Verastem, Inc.
Form 10-Q
May 14, 2012

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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended March 31, 2012

OR

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

For the transition period from to
Commission file number: 001-35409

Verastem, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**27-3269467**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification Number)
215 First Street, Suite 440 Cambridge, MA	**02142**
(Address of principal executive offices)	(Zip Code)

(617) 252-9300
(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 for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ý 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 (§232.405 of this chapter) during the preceding 12 months

(or for such shorter period that the registrant was required to submit and post such files). Yes ý 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 Exchange Act.

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

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

As of May 4, 2012 there were 21,059,116 shares of Common Stock, $0.0001 par value per share, outstanding.

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FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Quarterly Report on Form 10-Q, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward looking statements. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements in this Quarterly Report on Form 10-Q include, among other things, statements about:

- our ongoing and planned discovery and development of drugs targeting cancer stem cells;
- our expectations regarding the role of cancer stem cells in tumor recurrence and metastasis;
- the potential advantages of our proprietary technology;
- our ability to acquire or in-license any compounds or product candidates from third parties that we identify using our proprietary technology or otherwise;
- our plans to develop and commercialize our product candidates and companion diagnostics;
- our ability to establish and maintain collaborations;
- the timing of and our ability to obtain and maintain regulatory approvals for our product candidates;
- the rate and degree of market acceptance and clinical utility of our products;
- our intellectual property position;
- our expectations regarding the use of proceeds from our initial public offering; and
- our estimates regarding expenses, future revenues, capital requirements and needs for additional financing.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Quarterly Report on Form 10-Q, particularly in the "Risk Factors" section, that could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make.

You should read this Quarterly Report on Form 10-Q and the documents that we have filed as exhibits to the Quarterly Report on Form 10-Q completely and with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as

required by law.

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PART I FINANCIAL INFORMATION

Item 1. Financial Statements (Unaudited).

Verastem, Inc.

(A development stage company)

CONDENSED BALANCE SHEETS

(unaudited)

(in thousands, except per share amounts)

	March 31, 2012	December 31, 2011
Assets		
Current assets:		
Cash and cash equivalents	$ 25,470	$ 20,954
Short-term investments	15,597	26,857
Prepaid expenses and other current assets	490	130
Total current assets	41,557	47,941
Property and equipment, net	793	709
Long-term investments	68,269	8,994
Other assets		1,307
Restricted cash	86	86
Total assets	$ 110,705	$ 59,037
Liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable	$ 1,936	$ 2,273
Accrued expenses	1,001	873
Total current liabilities	2,937	3,146
Deferred rent	66	74
Liability for shares subject to repurchase	27	36
Obligation to issue warrant		406
Series A redeemable convertible preferred stock, $0.0001 par value; no shares and 16,000 shares authorized, issued and outstanding at March 31, 2012 and December 31, 2011, respectively		15,939
Series B redeemable convertible preferred stock, $0.0001 par value; no shares and 16,025 shares authorized, issued and outstanding at March 31, 2012 and December 31, 2011, respectively		31,948
Series C redeemable convertible preferred stock, $0.0001 par value; no shares and 9,068 shares authorized, issued and outstanding at March 31, 2012 and December 31, 2011, respectively		20,254
Stockholders' equity (deficit)		
Preferred stock, $0.0001 par value; 5,000 shares authorized; none issued		
Common stock, $0.0001 par value; 100,000 and 53,093, shares authorized at March 31, 2012 and December 31, 2011, respectively, 19,791 and 1,559 shares issued and outstanding at March 31, 2012 and December 31, 2011, respectively	2	1
Additional paid-in capital	129,056	1,702
Accumulated other comprehensive loss	(45)	(2)
Deficit accumulated during the development stage	(21,338)	(14,467)
Total stockholders' equity (deficit)	107,675	(12,766)

Total liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)	$ 110,705	$ 59,037

See accompanying notes.

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Verastem, Inc.

(A development stage company)

CONDENSED STATEMENTS OF COMPREHENSIVE LOSS

(unaudited)

(in thousands, except per share amounts)

	Three months ended, March 31,		Period from August 4, 2010 (inception) to March 31, 2012
	2012	2011	
Operating expenses:			
Research and development	$ 4,803	$ 675	$ 15,086
General and administrative	2,125	471	6,324
Total operating expenses	6,928	1,146	21,410
Loss from operations	(6,928)	(1,146)	(21,410)
Interest income	57		72
Net loss	(6,871)	(1,146)	(21,338)
Accretion of preferred stock	(6)	(4)	(40)
Net loss applicable to common stockholders	$ (6,877)	$ (1,150)	$ (21,378)
Net loss per share applicable to common stockholders basic and diluted	$ (0.47)	$ (1.06)	$ (6.62)
Weighted-average number of common shares used in net loss per share applicable to common stockholders basic and diluted	14,693	1,089	3,228
Comprehensive loss	$ (6,914)	$ (1,146)	$ (21,383)

See accompanying notes.

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Verastem, Inc.

(A development stage company)

CONDENSED STATEMENTS OF CASH FLOWS

(unaudited)

(in thousands)

	Three months ended March 31,		Period from August 4, 2010 (inception) to March 31, 2012
	2012	2011	
Operating activities			
Net loss	$ (6,871)	$ (1,146)	$ (21,338)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	46		129
Stock-based compensation expense	1,529	54	3,216
Common stock issued in exchange for license			46
Obligation to issue a warrant in exchange for license			439
Change in fair value of obligation to issue warrant	431		398
Changes in operating assets and liabilities:			
Prepaid expenses and other current assets	(360)	(8)	(490)
Accounts payable	(337)	134	1,936
Accrued expenses and deferred rent	667	99	1,067
Net cash used in operating activities	(4,895)	(867)	(14,597)
Investing activities			
Purchases of property and equipment	(130)		(923)
Purchases of investments	(72,758)		(108,609)
Maturities of investments	24,700		24,700
Increase in restricted cash			(86)
Net cash used in investing activities	(48,188)		(84,918)
Financing activities			
Proceeds from issuance of redeemable convertible preferred stock			68,107
Net proceeds from the issuance of common stock and restricted common stock	57,599		56,878
Net cash provided by financing activities	57,599		124,985
Increase (decrease) in cash and cash equivalents	4,516	(867)	25,470
Cash and cash equivalents at beginning of period	20,954	3,584	
Cash and cash equivalents at end of period	$ 25,470	$ 2,717	$ 25,470
Supplemental disclosure of non-cash financing activity			
Accretion of redeemable convertible preferred stock to redemption value	$ 6	$ 4	$ 40
Conversion of redeemable convertible preferred stock upon initial public offering	$ 68,148	$	$ 68,148
Reclassification of obligation to issue warrant from liabilities to equity	$ 837	$	$ 837

See accompanying notes.

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Verastem, Inc.

(A development stage company)

NOTES TO CONDENSED FINANCIAL STATEMENTS

1. Summary of significant accounting policies

Basis of presentation

The accompanying unaudited condensed financial statements of the Company have been prepared in accordance with accounting principles generally accepted in the United States ("GAAP") for interim financial reporting and as required by Regulation S-X, Rule 10-01. Accordingly, they do not include all of the information and footnotes required by GAAP for complete financial statements. In the opinion of management, all adjustments (including those which are normal and recurring) considered necessary for a fair presentation of the interim financial information have been included. When preparing financial statements in conformity with GAAP, the Company must make estimates and assumptions that affect the reported amounts of assets, liabilities, revenues, expenses and related disclosures at the date of the financial statements. Actual results could differ from those estimates. Additionally, operating results for the three months ended March 31, 2012 are not necessarily indicative of the results that may be expected for any other interim period or for the fiscal year ending December 31, 2012. For further information, refer to the financial statements and footnotes included in the Company's Annual Report on Form 10-K for the year ended December 31, 2011 as filed with the Securities and Exchange Commission ("SEC") on March 30, 2012.

Subsequent Events

In preparing the financial statements included in this Form 10-Q, the Company has evaluated all subsequent events that occurred after March 31, 2012 through the date of the filing of this Form 10-Q. On May 11, 2012, the Company acquired from S*Bio Pte Ltd, or S*Bio, compounds identified as dual inhibitors of PI3K and mTOR, including related patent rights. PI3K and mTOR are members of a network of proteins, or signaling pathway, that promotes cancer cell proliferation and survival. Under the agreement, the Company paid S*Bio an upfront fee of $350,000 and has agreed to pay S*Bio milestone payments of up to an aggregate of approximately $21.0 million upon the achievement of specified development and regulatory milestones. In addition, the Company agreed to pay to S*Bio tiered, low to mid single digit royalties as a percentage of annual net sales of each product containing an acquired compound as an ingredient. The obligation to pay royalties continues on a product by product and country by country basis until the expiration of all acquired patent rights covering the product in such country. If the Company obtains a license from a third party in order to commercialize an acquired compound contained in a product in a particular country, then the Company may deduct up to 50% of the amount paid to such third party from the royalty payments that Company owes to S*Bio for such product. The deduction is subject to specified limitations, including that in no event will any such deduction reduce a royalty payment owed to S*Bio by more than 50% as a result of all such deductions in the aggregate. The Company did not have any other material recognizable or unrecognizable subsequent events during this period.

Recent Accounting Pronouncements

In May 2011, the FASB issued ASU No. 2011-04, "Fair Value Measurement." This ASU clarifies the concepts related to highest and best use and valuation premise, blockage factors and other premiums and discounts, the fair value measurement of financial instruments held in a portfolio and of those instruments classified as a component of stockholders' deficit. The guidance includes enhanced disclosure requirements about recurring Level 3 fair value measurements, the use of nonfinancial assets

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Verastem, Inc.

(A development stage company)

NOTES TO CONDENSED FINANCIAL STATEMENTS (Continued)

1. Summary of significant accounting policies (Continued)

and the level in the fair value hierarchy of assets and liabilities not recorded at fair value. The Company adopted the ASU on January 1, 2012. The adoption did not have a significant impact on the financial statements.

In June 2011, the FASB issued ASU No. 2011-05, "Comprehensive Income." This ASU enhances comparability and transparency of other comprehensive income components. The guidance provides an option to present total comprehensive income, the components of net income and the components of other comprehensive income in a single continuous statement or two separate but consecutive statements. This ASU eliminates the option to present other comprehensive income components as part of the statement of changes in shareholder's equity (deficit). The Company adopted this ASU on January 1, 2012. The adoption of this standard did not have a significant impact on the financial statements, but it did impact the presentation of comprehensive loss in the financial statements.

2. Fair value of financial instruments

The Company is required to disclose information on all assets and liabilities reported at fair value that enables an assessment of the inputs used in determining the reported fair values. The fair value hierarchy is now established that prioritizes valuation inputs based on the observable nature of those inputs. The fair value hierarchy applies only to the valuation inputs used in determining the reported fair value of the investments and is not a measure of the investment credit quality. The hierarchy defines three levels of valuation inputs:

Level 1 inputs	Quoted prices in active markets for identical assets or liabilities
Level 2 inputs	Inputs other than quoted prices included within Level 1 that are observable for the asset or liability, either directly or indirectly
Level 3 inputs	Unobservable inputs that reflect the Company's own assumptions about the assumptions market participants would use in pricing the asset or liability

The following table presents information about the Company's financial assets that have been measured at fair value at March 31, 2012 and indicates the fair value hierarchy of the valuation inputs utilized to determine such fair value (in thousands).

Description	Total	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
Financial assets				
Cash equivalents	$ 9,037	$ 9,037	$	$
Short-term investments	15,597		15,597	
Long-term investments	68,269		68,269	
Total financial assets	**$ 92,903**	**$ 9,037**	**$ 83,866**	**$**

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Verastem, Inc.

(A development stage company)

NOTES TO CONDENSED FINANCIAL STATEMENTS (Continued)

2. Fair value of financial instruments (Continued)

The following table presents information about the Company's financial assets that have been measured at fair value at December 31, 2011 and indicates the fair value hierarchy of the valuation inputs utilized to determine such fair value (in thousands).

Description	Total	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)
Financial assets				
Cash equivalents	$ 4,102	$ 3,102	$ 1,000	$
Short-term investments	26,857		26,857	
Long-term investments	8,994		8,994	
Total financial assets	**$ 39,953**	**$ 3,102**	**$ 36,851**	**$**
Financial liabilities				
Obligation to issue warrant	$ 406	$	$	$ 406
Total financial liabilities	**$ 406**	**$**	**$**	**$ 406**

The Company's cash equivalents and investments are comprised of money market accounts, government-sponsored enterprise securities and commercial paper of publicly traded companies secured by the U.S. government. These investments have been initially valued at the transaction price and subsequently valued, at the end of each reporting period, utilizing third party pricing services or other market observable data. The pricing services utilize industry standard valuation models, including both income and market based approaches and observable market inputs to determine value. These observable market inputs include reportable trades, benchmark yields, credit spreads, broker/dealer quotes, bids, offers, current spot rates and other industry and economic events. The Company validates the prices provided by third party pricing services by reviewing their pricing methods and matrices, obtaining market values from other pricing sources, analyzing pricing data in certain instances and confirming that the relevant markets are active. After completing its validation procedures, the Company did not adjust or override any fair value measurements provided by the pricing services as of March 31, 2012.

In connection with the license with Poniard Pharmaceuticals Inc., the Company is obligated to issue a warrant to Poniard for the purchase of the Company's common stock upon the first patient dosing using a product licensed under the agreement with Poniard; such warrant will have a three year term from the date of issuance. Prior to an initial public offering, the exercise price of the warrant is equal to the fair value of the common stock on the date of the most recent preferred stock financing prior to the issuance of the warrant. Upon the completion of the Company's initial public offering in January 2012, the exercise price of the warrant is equal to the average closing price of the Company's common stock during the five trading days preceding the issuance of the warrant.

Prior to January 2012, the obligation to issue the warrant is a level 3 liability because its value measurement is based, in part, on significant inputs not observed in the market and reflects the Company's assumptions as to the expected warrant exercise price and the expected volatility of the Company's common stock. The obligation to issue the warrant was initially recorded at fair value and,

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Verastem, Inc.

(A development stage company)

NOTES TO CONDENSED FINANCIAL STATEMENTS (Continued)

2. Fair value of financial instruments (Continued)

prior to the Company's initial public offering, was revalued at the end of each reporting period, with the change in the fair value reported in research and development expense within the statement of operations. Upon the completion of the Company's initial public offering, the obligation to issue the warrant met the definition of an equity-classified derivative instrument since the remaining variable inputs were consistent with those in a fixed for fixed forward option agreement, and was therefore revalued as of January 26, 2012 with the change in fair value reported in research and development expense within the statement of operations. The fair value of the obligation to issue the warrant was then reclassified from liabilities to additional paid-in-capital on the Company's balance sheet. The Company will reassess the equity classification of the obligation to issue the warrant upon a change in facts and circumstances in future reporting periods.

As of December 31, 2011, the most recent issuance of the Company's Preferred Stock had been the issuance of the Series C Preferred Stock in November 2011. The Company estimated the value of the obligation to issue the warrant using a probability-weighted scenario analysis that incorporated the probability of the completion of an initial public offering. The analysis included estimating the stock price on each measurement date assuming that achievement of the milestone would be 100% probable. The estimated stock price contingent upon milestone achievement was determined by analyzing the post-announcement returns for public companies that progressed to Phase 1 clinical trials. The following inputs were used to determine the fair value of the obligation to issue the warrant:

		December 31, 2011	
	January 26, 2012	Non-IPO	IPO
Exercise price	$ 11.09	$ 6.86	$ 10.00
Estimated stock price contingent upon milestone achievement	$ 12.60	$ 3.22	$ 8.54
Expected term	4.0 years	4.1 years	4.1 years
Volatility	75%	70%	70%
Dividend yield	0.00%	0.00%	0.00%
Risk-free rate	0.54%	0.60%	0.60%
Probability of achieving milestone	80%	80%	80%
Probability of scenario	100%	20%	80%

As of December 31, 2011, the fair value of the obligation to issue the warrant was recorded at $406,000. As a result of the change in inputs to the valuation model, the fair value of the obligation to issue the warrant increased by $431,000 to $837,000 at January 26, 2012. Reasonable changes in the assumptions used to calculate the fair value of the obligation to issue the warrant would not result in significant changes in the fair value.

3. Investments

The Company's investments are classified as available-for-sale pursuant to Accounting Standards Codification (ASC) 320, *Investments Debt and Equity Securities*. The Company classifies investments available to fund current operations as current assets on its balance sheets. Investments are classified as long-term assets on the balance sheets if (i) the Company has the intent and ability to hold the

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Verastem, Inc.

(A development stage company)

NOTES TO CONDENSED FINANCIAL STATEMENTS (Continued)

3. Investments (Continued)

investments for a period of at least one year and (ii) the contractual maturity date of the investments is greater than one year.

Investments are carried at fair value with unrealized gains and losses included as a component of accumulated other comprehensive loss, until such gains and losses are realized. If a decline in the fair value is considered other-than-temporary, based on available evidence, the unrealized loss is transferred from other comprehensive loss to the statement of operations. There were no charges taken for other-than-temporary declines in fair value of short-term or long-term investments during the three months ended March 31, 2012 and 2011. Realized gains and losses are included in interest income in the statement of operations. There were no realized gains or losses recognized during the three months ended March 31, 2012 or 2011. The Company utilizes the specific identification method as a basis to determine the cost of securities sold.

The Company reviews investments for other-than-temporary impairment whenever the fair value of an investment is less than the amortized cost and evidence indicates that an investment's carrying amount is not recoverable within a reasonable period of time. To determine whether an impairment is other-than-temporary, the Company considers the intent to sell, or whether it is more likely than not that the Company will be required to sell, the investment before recovery of the investment's amortized cost basis. Evidence considered in this assessment includes reasons for the impairment, compliance with the Company's investment policy, the severity and the duration of the impairment and changes in value subsequent to year end. As of March 31, 2012, there were no investments with a fair value that was significantly lower than the amortized cost basis or any investments that had been in an unrealized loss position for a significant period.

Cash, cash equivalents and investments at March 31, 2012 and December 31, 2011 consist of the following (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
March 31, 2012				
Cash and cash equivalents:				
Cash and money market accounts	$ 25,470	$	$	$ 25,470
Investments:				
Government-sponsored enterprise securities (due within 1 year)	$ 2,500	$	$	$ 2,500
Government-sponsored enterprise securities (due within 1 2 years)	68,315	8	(54)	68,269
Commercial paper secured by the U.S. government (due within 1 year)	13,097			13,097
Total investments	$ 83,912	$ 8	$ (54)	$ 83,866
Total cash, cash equivalents, and investments	$ 109,382	$ 8	$ (54)	$ 109,336

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Verastem, Inc.

(A development stage company)

NOTES TO CONDENSED FINANCIAL STATEMENTS (Continued)

3. Investments (Continued)

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
December 31, 2011				
Cash and cash equivalents:				
Cash and money market accounts	$ 19,954	$	$	$ 19,954
Government-sponsored enterprise securities	1,000			1,000
Total cash and cash equivalents	$ 20,954	$	$	$ 20,954
Investments:				
Government-sponsored enterprise securities (due within 1 year)	$ 10,900	$ 2	$ (1)	$ 10,901
Government-sponsored enterprise securities (due within 1 2 years)	8,998	1	(5)	8,994
Commercial paper secured by the U.S. government (due within 1 year)	15,954	3	(1)	15,956
Total investments	$ 35,852	$ 6	$ (7)	$ 35,851
Total cash, cash equivalents, and investments	$ 56,806	$ 6	$ (7)	$ 56,805

4. Prepaid expenses and other current assets

Prepaid expenses and other current assets consist of the following (in thousands):

	March 31, 2012	December 31, 2011
Prepaid insurance	$ 311	$
Prepaid other expense	80	77
Interest receivable	99	53
	$ 490	$ 130

5. Accrued expenses

Accrued expenses consist of the following (in thousands):

	March 31, 2012	December 31, 2011
Compensation and related benefits	$ 367	$ 86
Contract research organizations	349	217
Professional fees	180	520
Other expenses	76	23
Deferred rent	29	27
	$ 1,001	$ 873

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Verastem, Inc.

(A development stage company)

NOTES TO CONDENSED FINANCIAL STATEMENTS (Continued)

6. Net loss per share

Basic and diluted net loss per common share is calculated by dividing net loss applicable to common stockholders by the weighted-average number of common shares outstanding during the period, without consideration for common stock equivalents. The Company's potentially dilutive shares, which include redeemable convertible preferred stock, outstanding stock options and unvested restricted stock are considered to be common stock equivalents and are only included in the calculation of diluted net loss per share when their effect is dilutive. The following table reconciles net loss to net loss applicable to common shareholders (in thousands, except per share data):

	Three Months ended		Period from August 4, 2010 (inception) to March 31, 2012
	March 31, 2012	March 31, 2011	
Net loss	$ (6,871)	$ (1,146)	$ (21,338)
Accretion of redeemable convertible preferred stock	(6)	(4)	(40)
Net loss applicable to common stockholders	$ (6,877)	$ (1,150)	$ (21,378)
Weighted-average number of common shares used in net loss per share applicable to common stockholders basic and diluted	14,693	1,089	3,228
Net loss per share applicable to common stockholders basic and diluted	$ (0.47)	$ (1.06)	$ (6.62)

The following potentially dilutive securities were excluded from the calculation of diluted net loss per share due to their anti-dilutive effect (in thousands):

	Three Months ended		Period from August 4, 2010 (inception) to March 31, 2012
	March 31, 2012	March 31, 2011	
Preferred stock		1,143	
Outstanding stock options	486	199	486
Unvested restricted stock	1,268	1,709	1,268
Unvested restricted stock units	600		600

7. Redeemable convertible preferred stock

In November 2010, the Company sold 4 million shares of Series A redeemable convertible preferred stock (Series A Preferred Stock) at a price of $1.00 per share for gross proceeds of $4 million. In accordance with the terms of the Series A Stock Purchase Agreement, the Company sold an additional 12 million shares at $1.00 per share in a second subsequent closing. The milestones necessary to achieve the subsequent closing were met in April 2011 and the Company sold 12 million shares of Series A Preferred Stock for gross proceeds of $12 million. The Company incurred approximately $79,000 of issuance costs as part of the first closing of the Series A Preferred Stock. No additional issuance costs were incurred as part of the second closing.

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Verastem, Inc.

(A development stage company)

NOTES TO CONDENSED FINANCIAL STATEMENTS (Continued)

7. Redeemable convertible preferred stock (Continued)

In July 2011, the Company sold approximately 16 million shares of series B redeemable convertible preferred stock (Series B Preferred Stock) at a price of $2.00 per share for gross proceeds of approximately $32 million. The Company incurred approximately $113,000 of issuance costs as part of the closing of the Series B Preferred Stock.

In November 2011, the Company sold approximately 9.1 million shares of Series C redeemable convertible preferred stock (Series C Preferred Stock) at a price of $2.25 per share for gross proceeds of $20.4 million. The Company incurred approximately $153,000 of issuance costs as part of the closing of the Series C Preferred Stock. The issuance costs associated with the Series A Preferred Stock, Series B Preferred Stock and Series C Preferred Stock (collectively, the Preferred Stock) were accreted through the earliest redemption date.

In connection with the Company's initial public offering, as discussed below, all shares of the Company's Preferred Stock were converted into 11,740,794 shares of common stock.

8. Common stock

Reverse Stock Split

In January 2012, the Company's board of directors and stockholders approved a one-for-3.5 reverse stock split of the Company's common stock. The reverse stock split became effective on January 10, 2012. All share and per share amounts in the financial statements have been retroactively adjusted for all periods presented to give effect to the reverse stock split, including reclassifying an amount equal to the reduction in par value to additional paid-in capital.

Initital Public Offering

In February 2012, the Company closed the initial public offering (IPO) of its common stock pursuant to a registration statement on Form S-1, as amended. An aggregate of 6,325,000 shares of common stock registered under the registration statement were sold at a public offering price of $10.00 per share, including the over-allotment option. Net proceeds of the IPO were $56.8 million.

9. Stock-based compensation

In December 2011, the Company adopted the 2012 Incentive Plan (the 2012 Plan). The 2012 Plan became effective upon the closing of the Company's IPO in February 2012. The 2012 Plan provides for the grant of incentive stock options, nonqualified stock options, stock appreciation rights, restricted stock awards, restricted stock units and other stock-based and cash awards. Upon effectiveness, the number of shares of common stock that are reserved under the 2012 Plan is the sum of 3,428,571 shares plus the number of shares available under the 2010 Plan. The number of shares reserved under the 2012 Plan is increased by the number of shares of common stock (up to a maximum of 571,242 shares) subject to outstanding awards under the 2010 Plan that expire, terminate or are otherwise surrendered, cancelled, forfeited or repurchased. The 2012 Plan includes an "evergreen provision" that allows for an annual increase in the number of shares of common stock available for issuance under the 2012 Plan. The annual increase will be added on the first day of each year beginning in 2013 and each subsequent anniversary until the expiration of the 2012 Plan, equal to the lowest of 1,285,714

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Verastem, Inc.

(A development stage company)

NOTES TO CONDENSED FINANCIAL STATEMENTS (Continued)

9. Stock-based compensation (Continued)

shares of common stock, 4.0% of the number of shares of common stock outstanding and an amount determined by the board of directors.

Restricted stock

A summary of the Company's nonvested restricted stock as of March 31, 2012 and changes during the three months ended March 31, 2012 is as follows (in thousands, except per share data):

	Shares	Weighted-average purchase price per share
Nonvested at December 31, 2011	1,435	$ 0.025
Vested	(167)	0.054
Nonvested at March 31, 2012	1,268	$ 0.021

As of March 31, 2012, there was $9.6 million of total unrecognized stock-based compensation expense related to non-vested restricted stock. The expense is expected to be recognized over a weighted average period 2.5 years.

A summary of the Company's nonvested restricted stock units (RSUs) as of March 31, 2012 and changes during the three months ended March 31, 2012 is as follows (in thousands, except per share data):

	Shares	Weighted-average grant date fair value
Nonvested at December 31, 2011		$
Granted	600	11.10
Nonvested at March 31, 2012	600	$ 11.10

As of March 31, 2012, there was $6.3 million of total unrecognized stock-based compensation expense related to non-vested RSUs granted under the 2012 Plan. The expense is expected to be recognized over a weighted-average period of 3.8 years.

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Verastem, Inc.

(A development stage company)

NOTES TO CONDENSED FINANCIAL STATEMENTS (Continued)

9. Stock-based compensation (Continued)

Stock options

A summary of the Company's stock option activity and related information follows (in thousands, except per share data):

	Shares	Weighted-average price per share	Weighted-average remaining contractual term (years)	Aggregate intrinsic value
Outstanding at December 31, 2011	405	$ 0.75	9.9	$ 176
Granted	81	11.10		
Outstanding at March 31, 2012	486	$ 2.47	9.2	$ 4,125
Exercisable at March 31, 2012	63	$ 0.28	8.7	$ 669
Vested and expected to vest at March 31, 2012	486	$ 2.47	9.2	$ 4,125

The fair value of each stock-based award is estimated on the grant date using the Black-Scholes option-pricing model using the following assumptions:

	Three Months ended March 31,	
	2012	2011
Risk-free interest rate	0.9-2.7%	2.0-2.7%
Dividend yield		
Volatility	69-72%	67-69%
Expected term (years)	5.3-6.1	6.1

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

You should read the following discussion and analysis of our financial condition and results of operations together with our financial statements and related notes appearing elsewhere in this quarterly report. The following discussion contains forward-looking statements that involve risks and uncertainties. Our actual results and the timing of certain events could differ materially from those anticipated in these forward-looking statements as a result of certain factors, including those discussed below and elsewhere in this quarterly report or in our annual report on Form 10-K.

OVERVIEW

We are a biopharmaceutical company focused on discovering and developing proprietary small molecule drugs targeting cancer stem cells in breast and other cancers along with proprietary companion diagnostics. A cancer stem cell is a particularly aggressive type of tumor cell, resistant to conventional cancer therapy, that we believe is an underlying cause of tumor recurrence and metastasis. Our scientific co-founders, Robert Weinberg, Ph.D., Eric Lander, Ph.D., and Piyush Gupta, Ph.D., have made discoveries that link the epithelial-to-mesenchymal transition, or EMT, to the emergence of cancer stem cells. This transition involves the transformation of one type of cancer cell into a more aggressive and drug resistant type of cancer cell. Building on these discoveries, our scientific co-founders developed proprietary technology to create a stable population of cancer stem cells that we use to screen for and identify small molecule compounds that target cancer stem cells.

We commenced active operations in the second half of 2010. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, acquiring and developing our technology, identifying potential product candidates and undertaking preclinical studies of our most advanced product candidates. To date, we have not generated any revenues and have financed our operations with net proceeds from the private placement of our preferred stock and our initial public offering. In February 2012, we completed an initial public offering of 6,325,000 shares of our common stock at a public offering price of $10.00 per share and received net proceeds of approximately $56.8 million, after deducting underwriting discounts and commissions and offering expenses.

As of March 31, 2012, we had a deficit accumulated during the development stage of $21.3 million. We had net losses of $6.9 million, $1.2 million and $21.3 million for the three months ended March 31, 2012 and 2011 and for the period from August 4, 2010 (inception) to March 31, 2012. We expect to incur significant expenses and increasing operating losses for the foreseeable future. We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development and later initiate clinical trials of, and seek marketing approval for, our product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution. Furthermore, we expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. Adequate additional financing may not be available to us on acceptable terms, or at all. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts. We will need to generate significant revenues to achieve profitability, and we may never do so.

CRITICAL ACCOUNTING POLICIES AND SIGNIFICANT JUDGMENTS AND ESTIMATES

We believe that several accounting policies are important to understanding our historical and future performance. We refer to these policies as "critical" because these specific areas generally require us to make judgments and estimates about matters that are uncertain at the time we make the

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estimate, and different estimates which also would have been reasonable could have been used, which would have resulted in different financial results.

The critical accounting policies we identified in our most recent Annual Report on Form 10-K for the fiscal year ended December 31, 2011 related to accrued research and development expenses and stock-based compensation. There were no changes to these critical accounting policies in the quarter ended March 31, 2012. It is important that the discussion of our operating results that follows be read in conjunction with the critical accounting policies disclosed in our Annual Report on Form 10-K, as filed with the SEC on March 30, 2012.

The Company has elected to follow the extended transition period guidance provided for in Securities Act Section 7(a)(2)(B) for complying with new or revised accounting standards. The Company will disclose the date on which adoption of such standards is required for non-emerging growth companies and the date on which the Company will adopt the recently issued accounting standards.

RESULTS OF OPERATIONS

Comparison of the Three Months ended March 31, 2012 and March 31, 2011

Research and development expense. Research and development expense for the three months ended March 31, 2012 (2012 Quarter) was $4.8 million compared to $675,000 for the three months ended March 31, 2011 (2011 Quarter). The $4.1 million increase from the 2011 Quarter to the 2012 Quarter is primarily related to an increase of $1.4 million in contract research organization expense for outsourced biology, chemistry and development services, an increase of $1.4 million for personnel costs, including stock-based compensation of $856,000, primarily due to increased headcount and higher fair value of our common stock, an increase of $482,000 in license fee expense primarily related to the revaluation of the obligation to issue the warrant to Poniard Pharmaceuticals and an increase of $315,000 for laboratory supplies.

General and administrative expense. General and administrative expense for the 2012 Quarter was $2.1 million compared to $471,000 for the 2011 Quarter. The $1.7 million increase from the 2011 Quarter to the 2012 Quarter principally resulted from an increase of $973,000 for personnel costs, including stock-based compensation of $620,000, primarily due to higher fair value of our common stock, an increase of $274,000 in professional fees primarily related to additional legal and accounting fees for being a publicly traded company, an increase of $157,000 in consulting fees and an increase of $96,000 in insurance costs primarily related to being a publicly traded company.

Interest income. Interest income increased to $57,000 for the 2012 Quarter from none for the 2011 Quarter. During the 2011 Quarter, our cash was deposited in non-interest bearing accounts.

Accretion of preferred stock. We recorded $6,000 of accretion in the 2012 Quarter reflecting the periodic accretion of issuance costs associated with our series A, series B and series C preferred stock compared to $4,000 in the 2011 Quarter reflecting the periodic accretion of issuance costs associated with our series A preferred stock.

LIQUIDITY AND CAPITAL RESOURCES

Sources of liquidity

To date, we have not generated any revenues. Since our inception in August 2010, we have financed our operations principally through private placements and public offerings of our equity securities, including our initial public offering, which we completed in February 2012. As of March 31, 2012, we had $109.3 million in cash and cash equivalents, short-term investments and long-term

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investments. We primarily invest our cash, cash equivalents and investments in a U.S. Treasury money market fund, government-sponsored enterprise securities and commercial paper.

Cash flows

Operating activities. The use of cash in all periods resulted primarily from our net losses adjusted for non-cash charges and favorable changes in the components of working capital. The significant increase in cash used in operating activities for the 2012 Quarter compared to the 2011 Quarter is due to an increase in research and development expenses as we increased our research and development headcount and increased spending on external research and development costs.

Investing activities. The cash used in investing activities for the 2012 Quarter reflects the net purchases of investments of $48.1 million and the purchase of $130,000 of property and equipment. There were no investing activities in the 2011 Quarter.

Financing activities. The cash provided by financing activities in the 2012 Quarter reflects the $56.8 million of net proceeds from our initial public offering less issuance costs paid in prior periods. There were no financing activities in the 2011 Quarter.

Funding requirements

We expect our existing cash, cash equivalents and investments will enable us to fund our current operating plan and capital expenditure requirements into 2016.

OFF-BALANCE SHEET ARRANGEMENTS

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined under Securities and Exchange Commission rules.

RECENTLY ADOPTED ACCOUNTING STANDARDS

Recent Accounting Pronouncements

In May 2011, the FASB issued ASU No. 2011-04, "Fair Value Measurement." This ASU clarifies the concepts related to highest and best use and valuation premise, blockage factors and other premiums and discounts, the fair value measurement of financial instruments held in a portfolio and of those instruments classified as a component of stockholders' deficit. The guidance includes enhanced disclosure requirements about recurring Level 3 fair value measurements, the use of nonfinancial assets and the level in the fair value hierarchy of assets and liabilities not recorded at fair value. We adopted the ASU on January 1, 2012. The adoption did not have a significant impact on the financial statements.

In June 2011, the FASB issued ASU No. 2011-05, "Comprehensive Income." This ASU enhances comparability and transparency of other comprehensive income components. The guidance provides an option to present total comprehensive income, the components of net income and the components of other comprehensive income in a single continuous statement or two separate but consecutive statements. This ASU eliminates the option to present other comprehensive income components as part of the statement of changes in shareholder's equity (deficit). We adopted this ASU on January 1, 2012. The adoption of this standard did not have a significant impact on the financial statements, but it did impact the presentation of comprehensive loss in the financial statements.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

We are exposed to market risk related to changes in interest rates. We had cash, cash equivalents and investments of $109.3 million as of March 31, 2012, consisting of cash, U.S. Treasury money market

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fund, government-sponsored enterprise securities and commercial paper. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because most of our investments are in short-term securities. Our available-for-sale securities are subject to interest rate risk and will fall in value if market interest rates increase. Due to the short-term duration most of our investment portfolio and the low risk profile of our investments, an immediate 100 basis point change in interest rates would not have a material effect on the fair market value of our portfolio.

We contract with CROs and contract manufacturers globally. We may be subject to fluctuations in foreign currency rates in connection with these agreements. Transactions denominated in currencies other than the functional currency are recorded based on exchange rates at the time such transactions arise. As of March 31, 2012, approximately $8,000 of our total liabilities were denominated in currencies other than the functional currency.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and Chief Operating Officer, evaluated the effectiveness of our disclosure controls and procedures as of March 31, 2012. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of March 31, 2012, our Chief Executive Officer and Chief Operating Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control Over Financial Reporting

No change in our internal control over financial reporting occurred during the fiscal quarter ended March 31, 2012 that has materially affected, or is reasonably likely to materially affect, the Company's internal control over financial reporting.

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PART II OTHER INFORMATION

Item 1. Legal Proceedings.

None.

Item 1A. Risk Factors.

RISKS RELATED TO OUR FINANCIAL POSITION AND NEED FOR ADDITIONAL CAPITAL

We have incurred significant losses since our inception. We expect to incur losses for the foreseeable future and may never achieve or maintain profitability.

Since inception, we have incurred significant operating losses. As of March 31, 2012, we had a deficit accumulated during the development stage of $21.3 million. To date, we have not generated any revenues and have financed our operations through private placements of our preferred stock and our initial public offering completed in February 2012. We have devoted substantially all of our efforts to research and development. We have not initiated clinical development of any product candidates and expect that it will be many years, if ever, before we have a product candidate ready for commercialization. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. The net losses we incur may fluctuate significantly from quarter to quarter. We anticipate that our expenses will increase substantially if and as we:

- continue our research and preclinical development of our product candidates;
- seek to identify additional product candidates that target cancer stem cells, or CSCs;
- acquire or in-license other products and technologies;
- initiate clinical trials for our product candidates;
- seek marketing approvals for our product candidates that successfully complete clinical trials;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- maintain, expand and protect our intellectual property portfolio;
- hire additional clinical, quality control and scientific personnel; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts.

To become and remain profitable, we must develop and eventually commercialize a product or products with significant market potential. This will require us to be successful in a range of challenging activities, including completing preclinical testing and clinical trials of our product candidates, obtaining marketing approval for these product candidates and manufacturing, marketing and selling those products for which we may obtain marketing approval. We may never succeed in these activities and, even if we do, may never generate revenues that are significant or large enough to achieve profitability. We are currently only in the preclinical testing stages for our most advanced product candidates and have

not yet completed preclinical development of any of our lead product candidates, VS-507, VS-4718 and VS-5095. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of the company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

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Our short operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

We are an early stage company. We commenced active operations in the second half of 2010. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, acquiring and developing our technology, identifying potential product candidates and undertaking preclinical studies of our most advanced product candidates. We completed our initial public offering in February 2012. All of our product candidates are still in preclinical development. We have not yet demonstrated our ability to initiate or successfully complete any clinical trials, including large-scale, pivotal clinical trials, obtain marketing approvals, manufacture a commercial scale product, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. It takes about ten to 15 years to develop one new medicine from the time it is discovered to when it is available for treating patients. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history.

In addition, as a new business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We will need to transition from a company with a research focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

We will need substantial additional funding. If we are unable to raise capital when needed, we would be forced to delay, reduce or eliminate our product development programs or commercialization efforts.

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development and later initiate clinical trials of, and seek marketing approval for, our product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts.

We expect our existing cash, cash equivalents and investments, will enable us to fund our current operating plan and capital expenditure requirements into 2016. Our future capital requirements will depend on many factors, including:

- the scope, progress, results and costs of compound discovery, preclinical development, laboratory testing and clinical trials for our product candidates;
- the extent to which we acquire or in-license other products and technologies;
- the costs, timing and outcome of regulatory review of our product candidates;
- the costs of future commercialization activities, including product sales, marketing, manufacturing and distribution, for any of our product candidates for which we receive marketing approval;
- revenue, if any, received from commercial sales of our product candidates, should any of our product candidates receive marketing approval;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims; and
- our ability to establish collaborations on favorable terms, if at all.

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Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of products that we do not expect to be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

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

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. We do not have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our existing stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our existing stockholders. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

If we raise additional funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

RISKS RELATED TO THE DISCOVERY, DEVELOPMENT AND COMMERCIALIZATION OF OUR PRODUCT CANDIDATES

Our approach to the discovery and development of product candidates that target CSCs is unproven, and we do not know whether we will be able to develop any products of commercial value.

Our scientific approach focuses on using proprietary technology to create a stable population of CSCs in the laboratory that we then use to screen for and identify product candidates targeting these CSCs. Research on CSCs is an emerging field and, consequently, there is ongoing debate regarding the existence of CSCs, whether the appropriate nomenclature to refer to these cells is cancer stem cells, tumor-initiating cells or another term and the importance of these cells as an underlying cause of tumor recurrence and metastasis.

Although there is general consensus that some cancer cells have tumor-initiating capacity, there also is some debate in the scientific community regarding the defining characteristics of these cells, which we call CSCs, and the origin of these cells. Some believe that normal adult stem cells mutate and transform into CSCs. Others believe that all cancer cells have tumor-initiating capabilities, these capabilities cannot be attributed to a factor intrinsic to a particular cell and, therefore, a definitive CSC cannot be isolated or targeted. We believe that the discovery by our scientific co-founders of the link between the epithelial-to-mesenchymal transition, or EMT, and the emergence of cancer stem cells is one way a cancer cell can transition to a CSC, but this view is not universally accepted.

Even if our beliefs regarding the existence, characteristics and function of CSCs are correct, any drugs that we develop may not effectively target CSCs. We do not believe that any drugs that target

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CSCs have been successfully developed to date for the treatment of cancer. If we are able to develop a drug that targets CSCs in preclinical studies, we may nonetheless not succeed in demonstrating safety and efficacy of the drug in human clinical trials. Our focus on using our proprietary technology to screen for and identify product candidates targeting CSCs may not result in the discovery and development of commercially viable drugs to treat cancer.

We may not be successful in our efforts to identify or discover additional potential product candidates.

A key element of our strategy is to identify and test additional compounds that target CSCs in a variety of different types of cancer. A significant portion of the research that we are conducting involves new compounds, new uses of existing compounds and new and unproven drug discovery methods, including our proprietary technology. The drug discovery that we are conducting using our EMT technology may not be successful in identifying compounds that are useful in treating cancer. Our research programs may initially show promise in identifying potential product candidates, yet fail to yield product candidates for clinical development for a number of reasons, including:

- the research methodology used may not be successful in identifying potential product candidates; or
- potential product candidates may, on further study, be shown to have harmful side effects or other characteristics that indicate that they are unlikely to be products that will receive marketing approval and achieve market acceptance.

In particular, because our EMT technology induces the EMT process to create a stable population of CSCs, it is possible that these stable CSCs may not react in precisely the same manner as naturally occurring CSCs when treated with a particular product candidate. As a result, a product candidate that shows initial promise in targeting our stable population of CSCs may not have the same effect on tumors with naturally occurring CSCs.

Research programs to identify new product candidates require substantial technical, financial and human resources. We may choose to focus our efforts and resources on a potential product candidate that ultimately proves to be unsuccessful.

If we are unable to identify suitable compounds for preclinical and clinical development, we will not be able to obtain product revenues in future periods, which likely would result in significant harm to our financial position and adversely impact our stock price.

We may not be successful in obtaining necessary rights to compounds and product candidates for our development pipeline through acquisitions and in-licenses.

Because we are screening a range of compounds, including compounds with proprietary rights held by third parties, for their activity against CSCs, the growth of our business will depend in significant part on our ability to acquire or in-license rights to these compounds. However, we may be unable to acquire or in-license any compounds or product candidates from third parties that we identify using our proprietary EMT technology or otherwise. The licensing and acquisition of proprietary compounds is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire compounds and product candidates that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities.

For example, although the Broad Institute has granted us a right of first negotiation for specified compounds and other intellectual property owned by the Broad Institute, we may be unable to negotiate a license within the specified time frame. If we are unable to do so, the Broad Institute may offer the intellectual property to other parties. In addition, the Whitehead Institute and affiliated parties have retained the right to use the EMT technology that we license from it for research, teaching

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and educational purposes and could seek to license to third parties any intellectual property rights that it discovers using the EMT technology while pursuing these purposes. Pursuant to our drug discovery platform license agreement with the Whitehead Institute, we will have an opportunity, subject to the Whitehead Institute's obligations under any third-party research funding agreements, to negotiate a license to any such intellectual property under the drug discovery platform license agreement that is developed or conceived on or prior to a specified date in Robert Weinberg's laboratory at the Whitehead Institute. Our failure to reach an agreement with either the Broad Institute or the Whitehead Institute for any applicable intellectual property could result in a third party acquiring the related rights.

In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire the relevant compound or product candidate on terms that would allow us to make an appropriate return on our investment.

In addition, we expect competition for acquisition and in-licensing product candidates that are attractive to us may increase in the future, especially if our approach of targeting CSCs gains greater scientific acceptance, which may mean fewer suitable opportunities for us as well as higher acquisition or licensing prices. If we are unable to successfully obtain rights to suitable compounds or product candidates, our business, financial condition and prospects for growth could suffer.

All of our product candidates are still in preclinical development. Preclinical testing and clinical trials of our product candidates may not be successful. If we are unable to commercialize our product candidates or experience significant delays in doing so, our business will be materially harmed.

We have invested a significant portion of our efforts and financial resources in the identification and preclinical development of drugs that target CSCs. Our ability to generate product revenues, which we do not expect will occur for many years, if ever, will depend heavily on the successful development and eventual commercialization of our product candidates. The success of our product candidates will depend on several factors, including the following:

- successful completion of preclinical studies and clinical trials;
- receipt of marketing approvals from applicable regulatory authorities;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity for our product candidates;
- launching commercial sales of the products, if and when approved, whether alone or in collaboration with others;
- acceptance of the products, if and when approved, by patients, the medical community and third-party payors;
- effectively competing with other therapies; and
- a continued acceptable safety profile of the products following approval.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our product candidates, which would materially harm our business.

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If clinical trials of our product candidates fail to demonstrate safety and efficacy to the satisfaction of regulatory authorities or do not otherwise produce positive results, we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our product candidates.

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. The outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products. For example, standard measures of clinical activity with respect to solid tumors, such as Response Criteria in Solid Tumors, or RECIST, measurement guidelines, which are based on gross changes in the size of tumor lesions, may not be sufficient to detect the targeting of CSCs by our product candidates.

We may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize our product candidates, including:

- regulators or institutional review boards may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we may have delays in reaching or fail to reach agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;
- clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development programs;
- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- we might have to suspend or terminate clinical trials of our product candidates for various reasons, including a finding that the participants are being exposed to unacceptable health risks;
- regulators or institutional review boards may require that we or our investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- the cost of clinical trials of our product candidates may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate; and
- our product candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators or institutional review boards to suspend or terminate the trials.

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If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical trials of our product candidates or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- be delayed in obtaining marketing approval for our product candidates;
- not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings, including boxed warnings;
- be subject to additional post-marketing testing requirements; or
- have the product removed from the market after obtaining marketing approval.

Our product development costs will also increase if we experience delays in testing or marketing approvals. We do not know whether any clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do and impair our ability to successfully commercialize our product candidates and may harm our business and results of operations.

If we experience delays or difficulties in the enrollment of patients in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.

We may not be able to initiate or continue clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these trials as required by the U.S. Food and Drug Administration, or FDA, or similar regulatory authorities outside the United States. In addition, many of our competitors have ongoing clinical trials for product candidates that treat the same indications as our product candidates, and patients who would otherwise be eligible for our clinical trials may instead enroll in clinical trials of our competitors' product candidates.

Patient enrollment is affected by other factors including:

- severity of the disease under investigation;
- eligibility criteria for the study in question;
- perceived risks and benefits of the product candidate under study;
- efforts to facilitate timely enrollment in clinical trials;
- patient referral practices of physicians;

the ability to monitor patients adequately during and after treatment; and

proximity and availability of clinical trial sites for prospective patients.

Our inability to enroll a sufficient number of patients for our clinical trials would result in significant delays or may require us to abandon one or more clinical trials altogether. Enrollment delays in our clinical trials may result in increased development costs for our product candidates, which would cause the value of our company to decline and limit our ability to obtain additional financing.

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If serious adverse or inappropriate side effects are identified during the development of our product candidates, we may need to abandon or limit our development of some of our product candidates.

All of our product candidates are still in preclinical development and their risk of failure is high. It is impossible to predict when or if any of our product candidates will prove effective or safe in humans or will receive marketing approval. If our product candidates are associated with undesirable side effects or have characteristics that are unexpected, we may need to abandon their development or limit development to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. Many compounds that initially showed promise in early stage testing for treating cancer have later been found to cause side effects that prevented further development of the compound.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus on research programs and product candidates that we identify for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

If we are unable to successfully develop companion diagnostics for our therapeutic product candidates, or experience significant delays in doing so, we may not realize the full commercial potential of our therapeutics.

We plan to develop companion diagnostics for our therapeutic product candidates. There has been limited success to date industry wide in developing these types of companion diagnostics. To be successful, we would need to address a number of scientific, technical and logistical challenges. We have only recently initiated development of companion diagnostics. We have limited experience in the development of diagnostics and may not be successful in developing appropriate diagnostics to pair with any of our therapeutic product candidates that receive marketing approval. Companion diagnostics are subject to regulation by the FDA and similar regulatory authorities outside the United States as medical devices and require separate regulatory approval prior to commercialization. Given our limited experience in developing diagnostics, we expect to rely in part on third parties for their design and manufacture. If we or any third parties that we engage to assist us, are unable to successfully develop companion diagnostics for our therapeutic product candidates, or experience delays in doing so:

- the development of our therapeutic product candidates may be adversely affected if we are unable to appropriately select patients for enrollment in our clinical trials;
- our therapeutic product candidates may not receive marketing approval if safe and effective use of a therapeutic product candidate depends on an *in vitro* diagnostic; and
- we may not realize the full commercial potential of any therapeutics that receive marketing approval if, among other reasons, we are unable to appropriately select patients who are likely to benefit from therapy with our drugs.

As a result, our business would be harmed, possibly materially.

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Even if any of our product candidates receive marketing approval, they may fail to achieve the degree of market acceptance by physicians, patients, healthcare payors and others in the medical community necessary for commercial success.

If any of our product candidates receive marketing approval, they may nonetheless fail to gain sufficient market acceptance by physicians, patients, healthcare payors and others in the medical community. For example, current cancer treatments like chemotherapy and radiation therapy are well established in the medical community, and doctors may continue to rely on these treatments. If our product candidates do not achieve an adequate level of acceptance, we may not generate significant product revenues and we may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- efficacy and potential advantages compared to alternative treatments;
- the ability to offer our products for sale at competitive prices;
- convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;
- sufficient third-party coverage or reimbursement; and
- the prevalence and severity of any side effects.

If, in the future, we are unable to establish sales and marketing capabilities or enter into agreements with third parties to sell and market our product candidates, we may not be successful in commercializing our product candidates if and when they are approved.

We do not have a sales or marketing infrastructure and have no experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for any approved product, we must either develop a sales and marketing organization or outsource these functions to third parties. In the future, we may choose to build a focused sales and marketing infrastructure to market or co-promote some of our product candidates if and when they are approved.

There are risks involved with both establishing our own sales and marketing capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force is expensive and time consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize our products on our own include:

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe any future products;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and

unforeseen costs and expenses associated with creating an independent sales and marketing organization.

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If we enter into arrangements with third parties to perform sales, marketing and distribution services, our product revenues or the profitability of these product revenues to us are likely to be lower than if we were to market and sell any products that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell and market our product candidates or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates.

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.

The development and commercialization of new drug products is highly competitive. We face competition with respect to our current product candidates, and will face competition with respect to any product candidates that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of the disease indications for which we are developing our product candidates. Some of these competitive products and therapies are based on scientific approaches that are the same as or similar to our approach, and others are based on entirely different approaches. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

We are developing our product candidates for the treatment of cancer. There are a variety of available therapies marketed for cancer. In many cases, these drugs are administered in combination to enhance efficacy. Some of these drugs are branded and subject to patent protection, and others are available on a generic basis. Many of these approved drugs are well established therapies and are widely accepted by physicians, patients and third-party payors. Insurers and other third-party payors may also encourage the use of generic products. We expect that if our product candidates are approved, they will be priced at a significant premium over competitive generic products. This may make it difficult for us to achieve our business strategy of using our product candidates in combination with existing therapies or replacing existing therapies with our product candidates.

There are also a number of products in clinical development by third parties to treat cancer by targeting CSCs. These companies include divisions of large pharmaceutical companies, including Astellas Pharma US, Inc., Sanofi-Aventis US LLC, GlaxoSmithKline plc, Boehringer Ingelheim GmbH, Pfizer Inc. and others. There are also biotechnology companies of various sizes that are developing therapies against CSCs, including OncoMed Pharmaceuticals, Inc., Boston Biomedical, Inc. and Stemline Therapeutics, Inc. Our competitors may develop products that are more effective, safer, more convenient or less costly than any that we are developing or that would render our product candidates obsolete or non-competitive. In addition, our competitors may discover biomarkers that more efficiently measure CSCs than our methods, which may give them a competitive advantage in developing potential products. Our competitors may also obtain marketing approval from the FDA or other regulatory authorities for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market.

Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated

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among a smaller number of our competitors. Smaller and other early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

Even if we are able to commercialize any product candidates, the products may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives, which would harm our business.

The regulations that govern marketing approvals, pricing and reimbursement for new drug products vary widely from country to country. In the United States, recently passed legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval.

Our ability to commercialize any products successfully also will depend in part on the extent to which reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that reimbursement will be available for any product that we commercialize and, if reimbursement is available, the level of reimbursement. Reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. Obtaining reimbursement for our products may be particularly difficult because of the higher prices often associated with drugs administered under the supervision of a physician. If reimbursement is not available or is available only to limited levels, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval.

There may be significant delays in obtaining reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the drug is approved by the FDA or similar regulatory authorities outside the United States. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Third-party payors often rely upon Medicare coverage policy and payment

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limitations in setting their own reimbursement policies. Our inability to promptly obtain coverage and profitable payment rates from both government-funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of any products that we may develop.

We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical trials and will face an even greater risk if we commercially sell any products that we may develop. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

decreased demand for any product candidates or products that we may develop;

injury to our reputation and significant negative media attention;

withdrawal of clinical trial participants;

significant costs to defend the related litigation;

substantial monetary awards to trial participants or patients;

loss of revenue; and

the inability to commercialize any products that we may develop.

We currently hold $3.0 million in product liability insurance coverage in the aggregate, with a per incident limit of $3.0 million, which may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage when we begin clinical trials or the commercialization of our product candidates, if ever. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological, hazardous or radioactive materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations

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may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

RISKS RELATED TO OUR DEPENDENCE ON THIRD PARTIES

We expect to depend on collaborations with third parties for the development and commercialization of our product candidates. If those collaborations are not successful, we may not be able to capitalize on the market potential of these product candidates.

We may seek third-party collaborators for the development and commercialization of our product candidates. Our likely collaborators for any collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. If we do enter into any such arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements.

Collaborations involving our product candidates would pose the following risks to us:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborator's strategic focus or available funding or external factors such as an acquisition that diverts resources or creates competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or product candidates if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- a collaborator with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;
- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our proprietary information or expose us to potential litigation;
- disputes may arise between the collaborators and us that result in the delay or termination of the research, development or commercialization of our products or product candidates or that result in costly litigation or arbitration that diverts management attention and resources; and
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.

Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If a present or future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program could be delayed, diminished or terminated.

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If we are not able to establish collaborations, we may have to alter our development and commercialization plans.

Our drug development programs and the potential commercialization of our product candidates will require substantial additional cash to fund expenses. For some of our product candidates, we may decide to collaborate with pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate. We may also be restricted under existing license agreements from entering into agreements on certain terms with potential collaborators. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of such product candidate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue.

We expect to rely on third parties to conduct our clinical trials and some aspects of our compound formulation research and preclinical testing, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials, research or testing.

We do not plan to independently conduct all aspects of clinical trials of our product candidates. In addition, we do not expect to independently conduct all aspects of our compound formulation research or preclinical testing of our product candidates. We expect to rely on third parties, such as contract research organizations, clinical data management organizations, medical institutions and clinical investigators, to conduct our clinical trials. We currently rely and expect to continue to rely on third parties to conduct some aspects of our compound formulation research and preclinical testing. For example, we currently rely on third parties in the development of various formulations of VS-507, VS-4718 and VS-5095. We cannot finish preclinical testing and initiate clinical trials of these product candidates until the development of a formulation is complete. Any of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements, it would delay our product development activities.

Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibilities. For example, we will remain

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responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with standards, commonly referred to as Good Clinical Practices, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. We also are required to register ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates.

We also expect to rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our product candidates or commercialization of our products, producing additional losses and depriving us of potential product revenue.

We contract with third parties for the manufacture of our product candidates for preclinical testing and expect to continue to do so for clinical trials and for commercialization. This reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or products or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.

We do not have any manufacturing facilities or personnel. We currently rely, and expect to continue to rely, on third-party manufacturers for the manufacture of our product candidates for preclinical testing, other than small amounts of compounds that we may synthesize ourselves for such purpose. To date, we have obtained starting materials for our supply of the cGMP bulk drug substance for our product candidates from one third-party manufacturer. We do not have a long term supply agreement with this third-party manufacturer, and we purchase our required drug supply on a purchase order basis.

We expect to rely on third-party manufacturers or third-party collaborators for the manufacture of our product candidates for clinical trials and for commercial supply of any of these product candidates for which we or our collaborators obtain marketing approval. We may be unable to establish any agreements with third-party manufacturers or to do so on acceptable terms. Even if we are able to establish agreements with third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

reliance on the third party for regulatory compliance and quality assurance;

the possible breach of the manufacturing agreement by the third party; and

the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us.

Third-party manufacturers may not be able to comply with current good manufacturing practices, or cGMP, regulations or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of product candidates or products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our products and harm our business and results of operations.

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Any products that we may develop may compete with other product candidates and products for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that might be capable of manufacturing for us.

Any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval. We do not currently have arrangements in place for redundant supply or a second source for bulk drug substance. If our current contract manufacturer cannot perform as agreed, we may be required to replace that manufacturer. Although we believe that there are several potential alternative manufacturers who could manufacture our product candidates, we may incur added costs and delays in identifying and qualifying any such replacement.

Our current and anticipated future dependence upon others for the manufacture of our product candidates or products may adversely affect our future profit margins and our ability to commercialize any products that receive marketing approval on a timely and competitive basis.

RISKS RELATED TO OUR INTELLECTUAL PROPERTY

If we fail to comply with our obligations under our intellectual property licenses with third parties, we could lose license rights that are important to our business.

We are a party to a number of intellectual property license agreements with third parties, including the Whitehead Institute and Poniard Pharmaceuticals, Inc., or Poniard, and expect to enter into additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, milestone payment, royalty, insurance and other obligations on us. For example, under our license agreements with the Whitehead Institute and Poniard, we are required to use commercially reasonable efforts to develop and commercialize licensed products under the agreement and to satisfy other specified obligations. If we fail to comply with our obligations under these licenses, our licensors may have the right to terminate these license agreements, in which event we might not be able to market any product that is covered by these agreements, or to convert the exclusive licenses to non-exclusive licenses, which could materially adversely affect the value of the product candidate being developed under these license agreements. Termination of these license agreements or reduction or elimination of our licensed rights may result in our having to negotiate new or reinstated licenses with less favorable terms. If the Whitehead Institute were to terminate its drug discovery platform license agreement with us for any reason, we would lose access to the EMT technology and the ability to use the stable population of CSCs for high-throughput screening. If Poniard were to terminate its license agreement with us for any reason, we would lose our rights to VS-4718 and VS-5095.

If we are unable to obtain and maintain patent protection for our technology and products, or if our licensors are unable to obtain and maintain patent protection for the technology or products that we license from them, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize technology and products similar or identical to ours, and our ability to successfully commercialize our technology and products may be adversely affected.

Our success depends in large part on our and our licensors' ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary technology and products. We and our licensors seek to protect our proprietary position by filing patent applications in the United States and abroad related to our novel technologies and products that are important to our business. To date, one U.S. patent has issued that covers an aspect of our proprietary technology, with claims covering certain methods of predicting the likelihood that a tumor will metastasize. However, no patents have issued that cover our proprietary EMT technology or our product candidates, and we cannot be certain that any patents will issue with claims that cover our proprietary EMT technology or product candidates.

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The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Moreover, in some circumstances, we do not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology or products that we license from third parties and are reliant on our licensors. For example, we do not control the prosecution of the patent applications licensed to us under our agreements with the Whitehead Institute or those patent applications owned by The Scripps Research Institute, or Scripps, licensed to us under our agreement with Poniard. Therefore, we cannot be certain that these patents and applications will be prosecuted and enforced in a manner consistent with the best interests of our business. If such licensors fail to maintain such patents, or lose rights to those patents, the rights we have licensed may be reduced or eliminated.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our and our licensors' patent rights are highly uncertain. Our and our licensors' pending and future patent applications may not result in patents being issued which protect our technology or products or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection.

The laws of foreign countries may not protect our rights to the same extent as the laws of the United States. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore we cannot be certain that we or our licensors were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions.

Assuming the other requirements for patentability are met, currently, in the United States, the first to make the claimed invention is entitled to the patent, while outside the United States, the first to file a patent application is entitled to the patent. In March 2013, the United States will transition to a first inventor to file system in which, assuming the other requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent. We may be subject to a third party preissuance submission of prior art to the U.S. Patent and Trademark Office, or become involved in opposition, derivation, reexamination, *inter parties* review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights.

Even if our owned and licensed patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner. For example, although we expect to file additional patent applications with respect to our product candidate VS-507 with claims directed to its formulation and method of use, patent protection is not available for composition of matter claims directed to its active pharmaceutical ingredient. Because VS-507 lacks composition of matter protection for its active pharmaceutical ingredient, competitors will be able to offer and sell products with the same active pharmaceutical

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ingredient so long as these competitors do not infringe any other patents that we may obtain covering this drug.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

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

Competitors may infringe our patents. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours is invalid or unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation proceeding could put one or more of our patents at risk of being invalidated or interpreted narrowly. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. In addition, our licensors may have rights to file and prosecute such claims and we are reliant on them.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing the proprietary rights of third parties. We have yet to conduct comprehensive freedom-to-operate searches to determine whether our use of certain of the patent rights owned by or licensed to us would infringe patents issued to third parties. We may become party to, or threatened with, future adversarial proceedings or litigation regarding intellectual property rights with respect to our products and technology, including interference proceedings before the U.S. Patent and Trademark Office. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future. If we are found to infringe a third party's intellectual property rights, we could be required to obtain a license from such third party to continue developing and marketing 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 were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. We could be forced, including by court order, to cease commercializing the infringing technology or product. In addition, we could be found liable for monetary damages. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

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For example, we are aware of a U.S. patent application filed by a third party almost one year after the priority date of the U.S. patent application filed by Scripps and licensed to us by Poniard, which has pending generic claims that, if issued as written, potentially cover VS-4718 and VS-5095. The third-party patent application also specifically discloses VS-4718. Although the Scripps patent application has a priority date that is earlier than the priority date of the third-party application, we cannot be sure which party was the first to make the claimed invention. Because the United States currently uses a first to invent standard to determine priority, if a patent issues under the third-party patent application covering the composition of matter of VS-4718 or VS-5095 and such third party was determined to be the first to make the claimed invention, we would need to obtain a license to the patented technology to commercialize VS-4718 or VS-5095 in the United States, which would cause us to incur licensing related costs. However, a license to this patent might not be available on commercially reasonable terms, or at all. Our failure to obtain a license to any such patent could delay or prevent our potential commercialization of VS-4718 or VS-5095 in the United States.

We may be subject to claims that our employees have wrongfully used or disclosed alleged trade secrets of their former employers.

Many of our employees were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these employees have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such employee's former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

Intellectual property litigation could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patents for some of our technology and products, we also rely on trade secrets, including unpatented know-how, technology and other proprietary information, to maintain our competitive position. We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our

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employees and consultants. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

RISKS RELATED TO REGULATORY APPROVAL OF OUR PRODUCT CANDIDATES AND OTHER LEGAL COMPLIANCE MATTERS

If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we will not be able to commercialize our product candidates, and our ability to generate revenue will be materially impaired.

Our product candidates and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by comparable authorities in other countries. Failure to obtain marketing approval for a product candidate will prevent us from commercializing the product candidate. We have not received approval to market any of our product candidates from regulatory authorities in any jurisdiction. We have only limited experience in filing and supporting the applications necessary to gain marketing approvals and expect to rely on third-party contract research organizations to assist us in this process. Securing FDA approval requires the submission of extensive preclinical and clinical data and supporting information to the FDA for each therapeutic indication to establish the product candidate's safety and efficacy. Securing FDA approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the FDA. Our product candidates may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use.

The process of obtaining marketing approvals, both in the United States and abroad, is expensive, may take many years if additional clinical trials are required, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. The FDA has substantial discretion in the approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate. Any marketing approval we ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable.

If we experience delays in obtaining approval or if we fail to obtain approval of our product candidates, the commercial prospects for our product candidates may be harmed and our ability to generate revenues will be materially impaired.

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Failure to obtain marketing approval in international jurisdictions would prevent our product candidates from being marketed abroad.

In order to market and sell our products in the European Union and many other jurisdictions, we or our third-party collaborators must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We or these third parties may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any market.

Any product candidate for which we obtain marketing approval could be subject to restrictions or withdrawal from the market and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products, when and if any of them are approved.

Any product candidate for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, advertising and promotional activities for such product, will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMP requirements relating to quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the product. The FDA closely regulates the post-approval marketing and promotion of drugs to ensure drugs are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we do not market our products for their approved indications, we may be subject to enforcement action for off-label marketing.

In addition, later discovery of previously unknown problems with our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing clinical trials;
- warning or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;

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fines, restitution or disgorgement of profits or revenue;

suspension or withdrawal of marketing approvals;

refusal to permit the import or export of our products;

product seizure; or

injunctions or the imposition of civil or criminal penalties.

Our relationships with customers and third-party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute our products for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations, include the following:

the federal healthcare anti-kickback statute 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 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;

the federal False Claims Act imposes 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, 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, as amended by the Health Information Technology for Economic and Clinical Health Act, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program and also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;

the federal false statements statute prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services;

the federal transparency requirements under the Health Care Reform Law requires manufacturers of drugs, devices, biologics and medical supplies to report to the Department of Health and Human Services information related to physician payments and other transfers of value and physician ownership and investment interests; and

analogous state laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, and some state laws require pharmaceutical companies to comply with the pharmaceutical

industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other health care providers or marketing expenditures.

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Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, exclusion from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we expect to do business are found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

Recently enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and affect the prices we may obtain.

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any product candidates for which we obtain marketing approval.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or Medicare Modernization Act, changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for drug purchases by the elderly and introduced a new reimbursement methodology based on average sales prices for physician administered drugs. In addition, this legislation provided authority for limiting the number of drugs that will be covered in any therapeutic class. Cost reduction initiatives and other provisions of this legislation could decrease the coverage and price that we receive for any approved products. While the Medicare Modernization Act applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from the Medicare Modernization Act may result in a similar reduction in payments from private payors.

More recently, in March 2010, President Obama signed into law the Health Care Reform Law, a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for health care and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. Effective October 1, 2010, the Health Care Reform Law revises the definition of "average manufacturer price" for reporting purposes, which could increase the amount of Medicaid drug rebates to states. Further, the new law imposes a significant annual fee on companies that manufacture or import branded prescription drug products. Substantial new provisions affecting compliance have also been enacted, which may affect our business practices with health care practitioners. We will not know the full effects of the Health Care Reform Law until applicable federal and state agencies issue regulations or guidance under the new law. Although it is too early to determine the effect of the Health Care Reform Law, the new law appears likely to continue the pressure on pharmaceutical pricing, especially under the Medicare program, and may also increase our regulatory burdens and operating costs.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's

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approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

RISKS RELATED TO EMPLOYEE MATTERS AND MANAGING GROWTH

Our future success depends on our ability to retain our president and chief executive officer and other key executives and to attract, retain and motivate qualified personnel.

We are highly dependent on Christoph Westphal, our President and Chief Executive Officer, Robert Forrester, our Chief Operating Officer, and Jonathan Pachter, our Vice President, Head of Research, as well as the other principal members of our management and scientific teams, including our scientific co-founders, Robert Weinberg, Eric Lander and Piyush Gupta. Although we have formal employment agreements with Robert Forrester and Jonathan Pachter, these agreements do not prevent them from terminating their employment with us at any time. We do not have an employment agreement with Christoph Westphal. We do not maintain "key person" insurance for any of our executives or other employees. The loss of the services of any of these persons could impede the achievement of our research, development and commercialization objectives.

In addition to his role as Chairman of the board of directors and President and Chief Executive Officer of our company, Dr. Westphal also serves as a general partner of Longwood Fund, LP, a venture capital investment fund and one of our principal stockholders. We and Dr. Westphal anticipate that he will transition to an executive Chairman role at our company in the future based on our having meaningfully advanced our discovery, research and development efforts, the overall growth of our company and our identifying and hiring a suitable successor. In connection with Dr. Westphal's transition to this role, we will need to recruit and hire a new principal executive officer. Our inability to hire a suitable executive to assume this position in a timely fashion could delay the execution of our business plans or disrupt our operations.

Recruiting and retaining qualified scientific, clinical, manufacturing and sales and marketing personnel will also be critical to our success. We may not be able to attract and retain these personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors, including our scientific co-founders, may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us.

We expect to expand our development, regulatory and future sales and marketing capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, regulatory affairs and sales and marketing. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The physical expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

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RISKS RELATED TO OUR COMMON STOCK

Our executive officers, directors and principal stockholders maintain the ability to control all matters submitted to stockholders for approval.

As of April 30, 2012, our executive officers, directors and stockholders who owned more than 5% of our outstanding common stock, in the aggregate, beneficially owned shares representing approximately 53% of our capital stock. As a result, if these stockholders were to choose to act together, they would be able to control all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would control the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of voting power could delay or prevent an acquisition of our company on terms that other stockholders may desire.

Provisions in our corporate charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our corporate charter and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

establish a classified board of directors such that not all members of the board are elected at one time;

allow the authorized number of our directors to be changed only by resolution of our board of directors;

limit the manner in which stockholders can remove directors from the board;

establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;

require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;

limit who may call stockholder meetings;

authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a "poison pill" that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and

require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

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If our stock price is volatile, our stockholders could incur substantial losses.

Our stock price is likely to be volatile. The stock market in general and the market for biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, our stockholders could incur substantial losses. The market price for our common stock may be influenced by many factors, including:

- the success of competitive products or technologies;
- results of clinical trials of our product candidates or those of our competitors;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates or products;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions; and
- the other factors described in this "Risk factors" section.

We incur significant costs as a result of operating as a newly-public company, and our management must devote substantial time to new compliance initiatives.

As a newly public company, we incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act of 2002 and rules subsequently implemented by the Securities and Exchange Commission and NASDAQ have imposed various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel must devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations have increased our legal and financial compliance costs and make some activities more time-consuming and costly.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be the source of gain for our stockholders.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be the sole source of gain for our stockholders for the foreseeable future.

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A significant portion of our total outstanding shares are restricted from immediate resale but may be sold into the market in the near future, which could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. As of April 30, 2012, we had outstanding 21,059,116 shares of common stock, of which 6,325,000 may be resold in the public market immediately without restriction, unless purchased by our affiliates or existing stockholders. The remaining 14,734,116 shares are currently restricted as a result of securities laws or lock-up agreements that extend until either July 24, 2012 or January 20, 2013. Moreover, holders of an aggregate of 11,740,794 shares of our common stock will have rights, subject to some conditions, to require us to file registration statements covering their shares or, along with holders of an additional 2,826,708 shares of our common stock, to include their shares in registration statements that we may file for ourselves or other stockholders. All shares of common stock that we may issue under our equity compensation plans can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates and lock-up agreements that extend until either July 24, 2012 or January 20, 2013.

We are an "emerging growth company," and our election to delay adoption of new or revised accounting standards applicable to public companies may result in our financial statements not being comparable to those of other public companies. As a result of this and other reduced disclosure requirements applicable to emerging growth companies, our common stock may be less attractive to investors.

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act, or JOBS Act, and may remain an emerging growth company for up to five years, until December 31, 2017, although if the market value of our common stock that is held by non-affiliates exceeds $700 million as of any June 30 before that time or if we have annual gross revenues of $1 billion or more in any fiscal year, we would cease to be an emerging growth company as of December 31 of the applicable year. For so long as we remain an emerging growth company, we are permitted and intend to rely on exemptions from certain reporting requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved.

Among other provisions, the JOBS Act provides that an emerging growth company can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act of 1933, as amended, or the Securities Act, for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We are electing to delay such adoption of new or revised accounting standards, and as a result, we may not comply with new or revised accounting standards on the relevant dates on which adoption of such standards is required for public companies that are not emerging growth companies. As a result of such election, our financial statements may not be comparable to the financial statements of other public companies.

We cannot predict whether investors will find our common stock less attractive because we will 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.

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

RECENT SALES OF UNREGISTERED SECURITIES

Set forth below is information regarding securities sold by us during the three months ended March 31, 2012, that were not registered under the Securities Act of 1933, as amended, or the Securities Act. Also included is the consideration, if any, received by us for the securities and information relating to the section of the Securities Act, or rule of the Securities and Exchange Commission, under which exemption from registration was claimed.

Issuances of securities

None.

Stock option and other equity awards

During the three months ended March 31, 2012, we issued to certain employees, directors and consultants options to purchase an aggregate of 80,714 shares of common stock at a weighted-average exercise price of $11.10 per share, no shares of restricted stock, and restricted stock units for an aggregate of 600,000 shares of our common stock.

The issuance of stock options and the common stock issuable upon the exercise of such options, and the grant of restricted stock units and the issuance of common stock issuable upon vesting of such restricted stock units, were issued pursuant to written compensatory plans or arrangements with our employees, directors and consultants, in reliance on the exemption from the registration requirements of the Securities Act provided by Rule 701 promulgated under the Securities Act or the exemption set forth in Section 4(2) under the Securities Act and Regulation D promulgated thereunder relative to transactions by an issuer not involving any public offering. All recipients either received adequate information about us or had access, through employment or other relationships, to such information.

PURCHASE OF EQUITY SECURITIES

We did not purchase any of our registered equity securities during the period covered by this Quarterly Report on Form 10-Q.

USE OF PROCEEDS FROM REGISTERED SECURITIES

In February 2012, we completed an initial public offering of 6,325,000 shares of our common stock at a public offering price of $10.00 per share for an aggregate offering price of $63.3 million. The offer and sale of all of the shares in the offering were registered under the Securities Act pursuant to a registration statement on Form S-1 (File No. 333-177677), which was declared effective by the SEC on January 26, 2012, and a registration statement on Form S-1 (File No. 333-179910) filed pursuant to Rule 462(b) of the Securities Act. UBS Securities LLC and Leerink Swann LLC acted as joint book-running managers of the offering and as representatives of the underwriters. Lazard Capital Markets LLC, Oppenheimer & Co. Inc. and Rodman & Renshaw, LLC acted as co-managers for the offering. The offering commenced on January 27, 2012 and did not terminate until the sale of all of the shares offered.

We received net proceeds from the offering of approximately $56.8 million, after deducting approximately $4.4 million in underwriting discounts and commissions and approximately $2.0 million in estimated offering expenses. None of the underwriting discounts and commissions or other offering expenses were incurred or paid to directors or officers of ours or their associates or to persons owning 10 percent or more of our common stock or to any affiliates of ours.

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As of March 31, 2012, we have used approximately $4.7 million of the net proceeds primarily to fund the preclinical development of VS-507, VS-4718 and VS-5095, to advance and expand the research and preclinical development of additional product candidates and companion diagnostics and for working capital, capital expenditures and other general corporate purposes. We have not used any of the net proceeds from the offering to make payments, directly or indirectly, to any director or officer of ours, or any of their associates, to any person owning 10 percent or more of our common stock or to any affiliate of ours. We have invested the balance of the net proceeds from the offering in a variety of capital preservation investments, including short-term, investment grade, interest bearing instruments and U.S. government securities. There has been no material change in our planned use of the balance of the net proceeds from the offering as described in our final prospectus filed with the SEC pursuant to Rule 424(b) under the Securities Act.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

None.

Item 5. Other Information.

On May 11, 2012, we acquired from S*Bio Pte Ltd, or S*Bio, compounds identified as dual inhibitors of PI3K and mTOR, including related patent rights. PI3K and mTOR are members of a network of proteins, or signaling pathway, that promotes cancer cell proliferation and survival. Published third-party research has reported that the PI3K/mTOR signaling pathway is up-regulated in a number of cancers, including breast cancer. In addition, we believe that the PI3K/mTOR signaling pathway plays an important role in cancer stem cell survival. Under the agreement, we paid S*Bio an upfront fee of $350,000 and have agreed to pay S*Bio milestone payments of up to an aggregate of approximately $21.0 million upon the achievement of specified development and regulatory milestones. In addition, we agreed to pay to S*Bio tiered, low to mid single digit royalties as a percentage of annual net sales of each product containing an acquired compound as an ingredient. The obligation to pay royalties continues on a product by product and country by country basis until the expiration of all acquired patent rights covering the product in such country. If we obtain a license from a third party in order to commercialize an acquired compound contained in a product in a particular country, then we may deduct up to 50% of the amount paid to such third party from the royalty payments that we owe to S*Bio for such product. This deduction is subject to specified limitations, including that in no event will any such deduction reduce a royalty payment that we owe to S*Bio by more than 50% as a result of all such deductions in the aggregate.

Item 6. Exhibits.

The exhibits filed as part of this Quarterly Report on Form 10-Q are set forth on the Exhibit Index, which Exhibit Index is incorporated herein by reference.

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SIGNATURES

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

VERASTEM, INC.

Date: May 14, 2012

By: /s/ CHRISTOPH WESTPHAL, M.D., PH.D.

Christoph Westphal, M.D., Ph.D.
President and Chief Executive Officer
(Principal executive officer)

Date: May 14, 2012

By: /s/ ROBERT FORRESTER

Robert Forrester
Chief Operating Officer
(Principal financial and accounting officer)

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EXHIBIT INDEX

31.1	Certification of Chief Executive Officer pursuant to Rules 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Certification of Chief Financial Officer pursuant to Rules 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1	Certification of Chief Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2	Certification of Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document

Submitted electronically herewith.

In accordance with Rule 406T of Regulation S-T, the XBRL related information in Exhibit 101 to this Quarterly Report on Form 10-Q is deemed not filed or part of a registration statement or prospectus for purposes of Sections 11 or 12 of the Securities Act, is deemed not filed for purposes of Section 18 of the Exchange Act, and otherwise is not subject to liability under these sections.