Verastem, Inc.
Form 10-K
March 30, 2012
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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

FORM 10-K

(Mark One)

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

For the fiscal year ended December 31, 2011

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 001-35403

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 No.)
215 First Street, Suite 440 Cambridge, Massachusetts	**02142**
(Address of principal executive offices)	(Zip Code)

Registrant s telephone number, including area code: **(617) 252-9300**

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Name of each exchange on which registered**
Common Stock, $0.0001 par value	**NASDAQ Global Market**

Securities registered pursuant to Section 12(g) of the Act: **None**

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. o Yes x No

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. o Yes x No

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. o Yes x No

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained, to the best of registrant s knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. x

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate website, 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). x Yes o No

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

Large accelerated filer o	Accelerated filer o
Non-accelerated filer x (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). o Yes x No

Aggregate market value of the voting and non-voting common equity held by non-affiliates of the registrant, based on the last sale price for such stock on March 15, 2012: $111,565,370. The registrant has provided this information as of March 15, 2012 because its common stock was not publicly traded as of the last business day of its most recently completed fiscal quarter.

The number of shares outstanding of the registrant s common stock as of March 15, 2012 was 21,059,116.

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FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Annual Report on Form 10-K, 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, expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements in this Annual Report on Form 10-K 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 Annual Report on Form 10-K, 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 Annual Report on Form 10-K and the documents that we have filed as exhibits to the Annual Report on Form 10-K 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

Item 1. Business

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 expect to initiate clinical trials with VS-507 and one of either VS-4718 or VS-5095 over the next 12 to 15 months.

Cancer is a group of diseases characterized by uncontrolled growth and spread of abnormal cells. The American Cancer Society estimates that in the United States in 2011, approximately 1.6 million new cases of cancer will be diagnosed and nearly 600,000 people will die from the disease. Current treatments for cancer include surgery, radiation therapy, chemotherapy, hormone therapy and targeted therapy. According to estimates by the National Institutes of Health, in the United States in 2010, the direct medical costs of cancer of all types exceeded $100 billion. IMS Health estimates that in the United States in 2010, approximately $22 billion was spent on drugs to treat cancer, representing the largest class of drug spending in the United States. Despite years of intensive research and clinical use, current treatments often fail to cure cancer.

We believe that a key reason for the ultimate failure of many current therapies to achieve a durable clinical response may be the presence of cancer stem cells, or CSCs, which are also sometimes referred to as tumor-initiating cancer cells, within tumors. CSCs have been identified in many types of cancer, including breast, pancreatic, colon, brain, lung and leukemia. Following many cancer treatments, the tumor can remain with a high percentage of CSCs and become more aggressive and resistant to further treatment. In addition, patients who relapse often develop metastatic disease in which the cancer spreads to other sites in the body. Tumor metastasis to critical organs is the cause of more than 90% of cancer deaths. We believe that it is the drug resistance and ability of CSCs to spread to other sites in the body that may be the root causes of these failed therapies. Accordingly, our mission is to develop drugs targeting CSCs that either in combination with other cancer treatments or alone can kill all of the cells comprising a tumor and, thus, create a durable clinical response.

We license our EMT technology from the Whitehead Institute for Biomedical Research, an affiliate of the Massachusetts Institute of Technology, or MIT, and the President and Fellows of Harvard College, or Harvard. We also have a first right to negotiate a license for additional related intellectual property from the Broad Institute, an affiliate of MIT and Harvard University, pursuant to which we have licensed intellectual property that relates to compounds identified using EMT technology that target CSCs. Using our proprietary technology, we can create a stable population of CSCs in the laboratory for use in rapid and automated assays, referred to as high-throughput screening, to enable discovery of novel drugs targeting these CSCs. We are using our discovery approach to identify a pipeline of small molecule compounds with the potential to target CSCs.

Our most advanced product candidates are VS-507, VS-4718 and VS-5095. We are currently evaluating these compounds in preclinical studies as potential therapies for breast and other cancers. We believe that these compounds may be especially beneficial as therapeutics in aggressive cancers with a high percentage of CSCs,

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such as triple negative breast cancer, or TNBC. TNBC is a type of breast cancer in which a high percentage of CSCs has been identified and that has a poorer prognosis and lower overall survival rate than other types of breast cancer.

Using our EMT technology, our scientific co-founders identified VS-507 as a drug candidate for killing breast CSCs. Their research on VS-507, which included an analysis of the effect of VS-507 on cell lines derived from TNBC, was published in the peer reviewed scientific journal *Cell* in 2009. Recently published third-party research has reported that VS-507 s activity may be mediated through the blockade of the Wnt/beta-catenin cell signaling pathway, a network of proteins that Dr. Weinberg described in 2011 in *Cell* as critical for the development and maintenance of CSCs. In mouse models of breast cancer, VS-507 treatment decreased biophysical or biochemical markers, referred to as biomarkers, of CSCs. In contrast, treatment in the same model with a standard chemotherapeutic agent, paclitaxel, increased biomarkers of CSCs.

We identified the CSC-targeted activity of VS-4718 and VS-5095 using our proprietary technology. In preclinical testing, these compounds were found to be potent and selective inhibitors of Focal Adhesion Kinase, or FAK, a protein which is involved in cell adhesion and motility. FAK expression is greater in many tumor types compared to normal tissue, particularly in cancers that have a high invasive and metastatic capability. In preclinical mouse models, both VS-4718 and VS-5095 demonstrated good oral bioavailability and pharmacokinetic and pharmacodynamic properties and effectively reduced both primary tumor growth and metastatic burden.

An important element of our business strategy is the development and use of proprietary, companion diagnostics in connection with the development of our therapeutic drug candidates. We plan to use these diagnostics as part of a personalized medicine approach to identify patients with aggressive cancers that have a high percentage of CSCs, which is the group that we believe will benefit most from our therapies. We also believe that these diagnostics may be used to monitor patients progress on therapy and aid physicians ongoing treatment decisions.

OUR MANAGEMENT TEAM AND SCIENTIFIC CO-FOUNDERS AND ADVISORS

Our experienced management team includes our President and Chief Executive Officer, Chairman and co-founder Christoph Westphal, M.D., Ph.D., our Chief Operating Officer, Robert Forrester, and our Vice President, Head of Research, Jonathan Pachter, Ph.D. Dr. Westphal has been involved in founding a number of biotechnology companies as chief executive officer, including Sirtris Pharmaceuticals, Inc., which was acquired by GlaxoSmithKline plc in 2008, as well as Alnylam Pharmaceuticals, Inc. and Momenta Pharmaceuticals, Inc. Dr. Westphal also co-founded Alnara Pharmaceuticals, Inc., which was acquired by Eli Lilly and Co. in 2010. Mr. Forrester has been the chief executive officer, chief operating officer and chief financial officer of both private and public life science companies, including Forma Therapeutics, Inc., CombinatoRx, Inc., now Zalicus Inc., and Coley Pharmaceutical Group, Inc., which was acquired by Pfizer Inc. in 2007. Dr. Pachter has over 20 years of experience in leading the discovery of small molecule and monoclonal antibody therapeutics for the treatment of cancer, most recently as the Senior Director of Cancer Biology at OSI Pharmaceuticals Inc., which was acquired by Astellas Pharma Inc. in 2010.

Our scientific co-founders are recognized leaders in the field of cancer biology. Robert Weinberg, Ph.D., Founding Member of the Whitehead Institute and Professor of Biology at MIT, has played a key role in identifying the genetic basis of cancer. Dr. Weinberg discovered the first tumor oncogene, the first tumor suppressor gene, the role of a protein related to the cell surface receptor HER2 in preclinical studies and the mechanisms underlying the formation of CSCs. Eric Lander, Ph.D., Founding Director of the Broad Institute,

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Professor of Biology at MIT and Professor of Systems Biology at Harvard Medical School, played a central role in the Human Genome Project. Piyush Gupta, Ph.D., Member of the Whitehead Institute and Assistant Professor of Biology at MIT, co-developed with Dr. Lander and Dr. Weinberg our proprietary EMT technology for use in the identification of drugs targeting CSCs and a genetic expression signature, useful as a biomarker, to monitor the effect of treatment.

Our management team is supported by our scientific advisory board comprised of leading academic and industry scientists. Our scientific advisory board consists of:

Scientific advisory board	
Robert Weinberg, Ph.D. *Scientific co-founder*	Founding Member of the Whitehead Institute for Biomedical Research, Professor of Biology at the Massachusetts Institute of Technology and recipient of the 1997 National Medal of Science
Eric Lander, Ph.D. *Scientific co-founder*	Founding Director of the Broad Institute, Professor of Biology at the Massachusetts Institute of Technology and Professor of Systems Biology at Harvard Medical School
Piyush Gupta, Ph.D. *Scientific co-founder*	Member of the Whitehead Institute for Biomedical Research and Assistant Professor of Biology at the Massachusetts Institute of Technology
Julian Adams, Ph.D.	President of Research and Development of Infinity Pharmaceuticals, Inc., former Senior Vice President of Drug Discovery and Development of Millennium Pharmaceuticals, Inc. and co-inventor and co-developer of Velcade
José Baselga, M.D., Ph.D.	Chief of Hematology and Oncology at Massachusetts General Hospital, Associate Director of the Massachusetts General Hospital Cancer Center and Professor of Medicine at Harvard Medical School
George Daley, M.D., Ph.D.	Professor of Hematology and Oncology and Director of the Stem Cell Transplantation Program at Children s Hospital and Professor of Biological Chemistry and Molecular Pharmacology at Harvard Medical School
Peter Elliott, Ph.D.	Former Senior Vice President and Head of Research and Development of Sirtris Pharmaceuticals, Inc., former Vice President of Pharmacology and Drug Development of Millennium Pharmaceuticals, Inc. and co-developer of Velcade
Daniel Haber, M.D., Ph.D.	Director of the Massachusetts General Hospital Cancer Center and Professor of Medicine at Harvard Medical School
Joseph (Yossi) Schlessinger, Ph.D.	Chairman and Professor in the Department of Pharmacology at Yale School of Medicine
Phillip A. Sharp, Ph.D.	Institute Professor at the David H. Koch Institute for Integrative Cancer Research at the Massachusetts Institute of Technology and recipient of the 1993 Nobel Prize in Medicine and Physiology
Roger Tung, Ph.D.	President and Chief Executive Officer of Concert Pharmaceuticals, Inc., former Vice President of Drug Discovery of Vertex Pharmaceuticals, Inc. and co-inventor of Lexiva and Agenerase
Christopher Walsh, Ph.D.	Hamilton Kuhn Professor in the Department of Biological Chemistry and Molecular Pharmacology at Harvard Medical School
Eric Winer, M.D.	Director of the Breast Oncology Center at the Dana Farber Cancer Institute and Professor of Medicine at Harvard Medical School

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THE PROBLEM

The cancer death rate in the United States has only decreased modestly since the early 1990s. Cancer remains one of the world s most serious health problems and is the second most common cause of death in the United States after heart disease. The American Cancer Society estimates that in the United States in 2011, approximately 1.6 million new cases of cancer will be diagnosed and nearly 600,000 people will die from the disease. According to estimates by the National Institutes of Health, in the United States in 2010, the direct medical cost of cancer of all types exceeded $100 billion and the cancer type responsible for the highest individual disease costs was breast cancer at $16.5 billion. The following table sets forth the U.S. annual incidence, based on 2011 estimates from the American Cancer Society, and the prevalence, or the number of people in the United States who have been previously diagnosed with cancer, based on 2010 estimates from the National Cancer Institute, for select cancers in which CSCs have been implicated.

Cancer type	U.S. annual incidence	U.S. prevalence
Breast	230,480	2,645,621
Lung and bronchus	221,130	373,489
Colorectal	141,210	1,110,077
Leukemia	44,600	253,350
Pancreatic	44,030	34,657
Brain and other nervous system cancers	22,340	128,193

For tumors that have not yet metastasized and remain localized to the site of original tumor formation, current treatments for cancer can be effective in initially reducing tumor burden. However, for many forms of cancer, current treatments lack sufficient efficacy to achieve a durable clinical response. Following initial treatment, the tumor may recur at the same site or metastasize and spread to other sites in the body. The vast majority of patients who succumb to cancer are killed by tumors that have metastasized. This is illustrated by the information in the following table, which shows, according to the National Cancer Institute s *SEER Cancer Statistics Review, 2001-2007*, the reduction in five-year survival rate for breast cancer patients based on the stage of the disease at the time at which the disease is diagnosed. The percentage of patients diagnosed at each stage of disease, referred to as stage distribution, is included below for comparative purposes.

Breast cancer stage at diagnosis	Stage distribution(1)	Five-year relative survival rate
Localized (confined to primary site)	60%	98.6%
Regional (spread to regional lymph nodes)	33%	83.8%
Distant (cancer has metastasized)	5%	23.4%

(1) 2% of breast cancer cases were designated as unknown stage.

With the application of new technologies and key discoveries, we believe that we are now entering an era of cancer research characterized by a more sophisticated understanding of the biology of cancer. We believe that the discovery of CSCs and the role that they play in cancer development are important new insights that present the opportunity to develop more effective treatments.

Epithelial-to-mesenchymal transition

In most solid tumors, the cells that make up the tissue mass have a characteristic epithelial appearance. Epithelial cells generally have a multi-sided, uniform shape. Epithelial cells also have well-defined contact

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points with neighboring cells and a strong attachment to the underlying connective tissue that creates a framework for solid tumors in the body. Epithelial cells generally lack the ability to separate from these connection points to move, invade or metastasize into surrounding tissue or other sites in the body.

Epithelial cells can undergo a transformation to a different cell type, called mesenchymal cells, through a process called epithelial-to-mesenchymal transition, or EMT. In contrast with epithelial cells, mesenchymal cells have an elongated spindle shape, lack orderly contacts with neighboring cells and can survive without contact with a surface or connective tissue. The EMT process is a series of reprogramming events that normally operates during the development of tissues and organs prior to birth. However, the EMT process also can be appropriated by epithelial cancer cells that are referred to as carcinoma cells. When epithelial carcinoma cells residing in a solid tumor undergo the EMT process, the resulting mesenchymal cancer cells have the capability to invade through local barriers and metastasize to other sites in the body.

Another consequence of epithelial carcinoma cells undergoing the EMT process is that the resulting mesenchymal cancer cells have significantly increased resistance to current cancer treatments. Retrospective analyses of data from two Phase 3 clinical trials in lung cancer, one published in *Clinical Cancer Research* in 2005 and the other presented at the 2009 World Conference on Lung Cancer, revealed that patients with high expression of epithelial biomarkers responded better to the anti-cancer drug Tarceva in terms of both progression-free survival and overall survival than patients in the same two trials with low levels of epithelial biomarkers in their tumors. These results suggest that the mesenchymal cancer cell population, which lacks epithelial biomarkers, is resistant to these therapies. These clinical observations are consistent with preclinical studies published in *Cancer Research* in 2005 reporting that lung cancer cells expressing mesenchymal biomarkers appeared to be resistant to Tarceva and other targeted anti-cancer agents when transplanted into mice.

Cancer stem cells

We believe that CSCs, which are sometimes referred to as tumor-initiating cancer cells, are responsible for the initiation, metastasis and recurrence of many cancers. CSCs have the ability to:

- move freely and proliferate without attachment to other cells or surfaces;
- initiate a tumor;
- self-renew;
- produce other cancer cell types; and

- resist many current cancer treatments.

CSCs are often characterized by a distinctive set of biomarkers, which we believe may be a key to identifying patients with tumors that are likely to respond to therapies targeting CSCs.

CSCs may be more resistant to current cancer treatments than other types of cancer cells. Thus, as illustrated in the figure below, while current treatments may succeed at initially decreasing tumor burden, they may leave behind a population of CSCs that can regenerate tumors. Therefore, the presence of a mixture of CSCs and other types of cancer cells within a tumor may necessitate a therapeutic approach combining drugs that can kill both cell populations.

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The need to target CSCs may apply across the treatment of a broad range of cancers. CSCs have been isolated and characterized from many types of cancer, including breast, pancreatic, colon, brain, lung and leukemia. The CSCs isolated from each of these tumor types have been found to confer greater tumor-forming capability when transplanted into mice than other types of cancer cells from the same tumor.

Several specific signaling pathways have been implicated in CSC biology. The combined action *in vitro* of the TGF-beta and Wnt signaling pathways in the formation and survival of CSCs was described by Dr. Weinberg in *Cell* in 2011. Separately, FAK has been found to increase the metastatic capability of breast cancer cells following the EMT process.

CSCs from breast cancer have been characterized in several studies. For example, in a study conducted at the Baylor College of Medicine, breast cancer biopsies were taken from patients at the time of initial diagnosis and again following 12 weeks of treatment with docetaxel, a standard cancer chemotherapy widely used to treat breast cancer. The biopsies taken after 12 weeks of treatment showed increased expression of biomarkers for CSCs and an increased number of chemoresistant cells as compared to biopsies taken at the time of initial diagnosis. This result indicates that the CSC component of the tumor was relatively resistant to the chemotherapy. Moreover, it supports our belief that either a combination of treatments or a single therapy that can effectively target both CSCs and other types of cancer cells is critical to create a durable clinical response.

OUR SOLUTION

Our solution is to discover and develop a next generation of oncology therapeutics targeting cancer stem cells along with companion diagnostics. We believe that by developing therapeutics that target CSCs we can address the problem of cancer recurrence and metastasis and create a durable clinical response.

Our scientific co-founders at the Whitehead Institute and the Broad Institute made discoveries linking the activation of the EMT process in epithelial cancer cells to the emergence of CSCs. Their studies demonstrated that the EMT process can be activated *in vitro* by forcing a higher level of expression of genes that direct the EMT process or by eliminating key epithelial proteins. The mesenchymal cancer cells that emerge from this induced EMT process have the hallmarks of CSCs, including tumor-forming ability and increased resistance to chemotherapeutic drugs. Our solution utilizes proprietary technology based on the discovery linking the EMT process to the emergence of CSCs. We use this technology along with high-throughput screening methods to identify drugs targeting CSCs and develop companion diagnostics. To achieve a durable clinical response, we believe that it may be necessary to kill both CSCs and other types of cancer cells in a tumor, as illustrated in the

figure below, either with a combination of current cancer treatments and CSC-targeted drugs or a single therapeutic found to target both cancer cell populations.

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Our proprietary technology

A persistent problem in the discovery of drugs targeting CSCs is the difficulty of isolating large numbers of CSCs. Without such large numbers, the discovery of drugs targeting CSCs using high-throughput screening is extremely difficult. Moreover, when CSCs are isolated, they typically do not remain stable in culture. Instead, over a short period of time, CSCs convert into other types of cancer cells. To address this problem, our scientific co-founders developed proprietary technology based on the EMT process to create a stable population of CSCs that are suitable for use in high-throughput screening of small molecule compounds. These stable CSCs are similar to natural CSCs in that they are drug resistant and capable of forming new tumors.

We license our EMT technology from the Whitehead Institute. Through December 31, 2011, we and scientists at the Whitehead Institute and the Broad Institute had used our technology and high-throughput screening methods to evaluate the ability of over 320,000 compounds to kill CSCs. We hold exclusive license rights to compounds and uses identified under our agreement with the Whitehead Institute. We also hold a right of first negotiation to compounds identified under our agreement with the Broad Institute and have licensed one such compound pursuant to that right.

To identify compounds that are selective for CSCs, we grow cancer non-stem cells in the laboratory and then induce the EMT process to create a stable population of CSCs. As illustrated in the figure below, we then screen compounds to assess their ability to kill the CSCs. Because these CSCs are stable in culture, the screening process can be conducted using high-throughput technology on a large number and wide variety of small molecule compound libraries. These compound libraries include new chemical entities, or NCEs, approved drugs and compounds that are in preclinical and clinical development. We then profile the compounds that are identified as selective for CSCs using additional assays to identify suitable clinical candidates.

Biomarkers and diagnostics

Because of the high level of toxicity of traditional chemotherapies and the variability in response of tumors to these treatments, it is critically important to get the right cancer drug to the right patient. As a result, the oncology field has been at the forefront of developing diagnostics to select patients who may benefit from specific therapies, which is sometimes referred to as personalized medicine. We plan to build on the methods

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incorporated in our EMT technology to develop diagnostics designed to enhance our ability to deliver the right drug to the right patient.

In particular, we are identifying specific protein and gene biomarkers that are either present or conspicuously absent in CSCs. We are also developing panels of multiple biomarkers, which we believe may be more effective at identifying CSCs than individual biomarkers alone. We believe that our diagnostics will enable us to identify patients with aggressive cancers that have a high percentage of CSCs. We further believe that these patients are the most likely to benefit from our drug candidates. By screening to identify these patients, we expect to be able to select appropriate patients for enrollment in our clinical trials and ultimately, if we obtain marketing approval, patients who are likely to respond to our therapies. We also plan to use these diagnostics to measure the selective killing of CSCs by our drug candidates as one of the ways of determining their efficacy.

We expect that our use of proprietary diagnostics may accelerate the clinical development process for our drug candidates by enabling smaller, targeted trials. We believe that use of these diagnostics may provide early, objective signals of drug activity to guide us to optimal dosing and the sequencing of agents more quickly. We also believe that this approach may ultimately enable physicians to identify patients who are likely to benefit most from these therapies and make better clinical decisions during therapy.

We are working on companion diagnostics for our therapeutic programs based on both in-licensed and internally developed technology and science. We believe that augmenting our internal capabilities with external collaborations with experienced third parties can reduce development risk and accelerate our progress in this field.

OUR STRATEGY

We believe that a key reason for the failure of many current cancer treatments is that they fail to kill CSCs, which we believe are responsible for the initiation, metastasis and recurrence of many cancers. Our goal is to build a leading biopharmaceutical company focused on the discovery, development and, ultimately, commercialization of novel drugs and companion diagnostics targeting CSCs. Key elements of our strategy to achieve this goal are:

- *Continue to screen and identify small molecules that target CSCs.* We plan to use our proprietary EMT technology and high-throughput screening methods to identify additional compounds that target CSCs. We also plan to further optimize these agents through medicinal chemistry as necessary to create drug candidates.

- *In-license rights to additional compounds to expand our pipeline of candidates that target CSCs.* We plan to pursue the acquisition or in-license from third parties of rights to additional compounds that target CSCs, including compounds that are in preclinical and clinical development. We believe that our approach of identifying drug candidates from external sources at various stages of development to supplement our internal programs may allow us to initiate clinical development of a diverse pipeline of compounds more quickly than if we were to focus solely on internally developed NCEs.

- *Rapidly advance our drug candidates into clinical development.* We expect to initiate clinical trials with VS-507 and one of either VS-4718 or VS-5095 over the next 12 to 15 months. Our goal is to initiate clinical development of a number of additional therapeutic candidates over the next several years.

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- *Develop diagnostics for therapeutic products targeting CSCs.* We plan to develop companion diagnostic products to support our therapeutic product candidates. We believe that use of these diagnostics may aid in the selection of patients for enrollment in our clinical trials and, if we obtain marketing approval, patients who are most likely to benefit from therapy with our drugs. We also believe that these diagnostics may be used to monitor patients progress on therapy and aid physicians ongoing treatment decisions.

- *Collaborate selectively to augment and accelerate development and commercialization.* We may seek third-party collaborators for the development and commercialization of our product candidates. In particular, we may enter into third-party arrangements for target oncology indications in which our potential collaborator has particular expertise or for which we need access to additional research, development or commercialization resources.

- *Maintain scientific leadership in the CSC field.* We plan to continue to conduct research in the field of EMT and CSCs to further our understanding of the underlying biology of cancer progression and metastasis. We also plan to continue fostering relationships with top scientific advisors, researchers and physicians. We believe that investing in the recruitment of exceptional advisors, employees and management is critical to leadership in the CSC field.

OUR PRODUCT CANDIDATES

Using our proprietary technology and high-throughput screening methods, we are evaluating compounds for their activity against CSCs in a way that we believe has not been previously possible. We are focused on the discovery and development of small molecules to expedite the path to human clinical trials and to allow flexibility in the design of molecules for optimized efficacy and safety regardless of the route of administration.

We intend to incorporate CSC-specific biomarkers into companion diagnostics for our product candidates for use in identifying patients whose tumors have a high percentage of CSCs and are likely to benefit from treatment. We may use this information to aid in the selection of patients for late stage clinical trials. We also plan to utilize these diagnostics to measure the effect that our product candidates have on CSCs in a tumor.

We are developing our product candidates for the treatment of breast cancer, initially triple negative breast cancer, and other cancers with a high percentage of CSCs. We believe that our product candidates target CSCs that have been implicated in aggressive cancers, metastasis and chemotherapeutic resistance. To enhance therapeutic benefit, we may also use our product candidates in combination with existing therapies in an effort to target both CSCs and other types of cancer cells.

BREAST CANCER

Overview

The National Cancer Institute estimated that in January 2008 there were approximately 2.6 million women in the United States with a history of breast cancer. Breast cancer is currently the second most frequently diagnosed and the second most deadly cancer among women in the United States. The American Cancer Society estimates that in the United States in 2011, approximately 230,500 new cases of invasive breast cancer will be diagnosed in women and approximately 39,500 women will die from the disease.

Breast cancers can be segregated into subtypes based upon the positive presence of three protein receptors:

- estrogen receptor, or ER;

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- progesterone receptor, or PR; and

- human epidermal growth factor receptor 2, or HER2.

Triple negative breast cancer, or TNBC, is a type of breast cancer that does not express any of these three receptors. According to results from a population-based study of the California Cancer Registry published by the American Cancer Society in 2007, approximately 15% of all breast cancers were classified as TNBC. In comparison with other breast cancers, TNBC tends to grow faster and has a higher rate of metastases. Furthermore, TNBC tends to recur more often than other subtypes of breast cancer. Patients with TNBC generally have a poorer prognosis and lower overall survival rate than patients with breast cancers that are positive for the hormone receptors ER and PR.

We believe that the natural disease progression of TNBC exhibits the key hallmarks of CSCs. Specifically, we believe that:

- TNBC is initially responsive to chemotherapy because chemotherapy kills the majority of cancer cells, but not the CSCs.

- TNBC returns more often than other types of breast cancer in part because there are CSCs that are not killed by current cancer treatments.

- The site of recurrence is often at another place in the body than the original tumor because the CSCs not killed are able to metastasize.

- The recurring tumor may be resistant to therapy because it contains a high percentage of CSCs.

We believe that our product candidates may be especially beneficial as therapeutics for the treatment of TNBC. We are currently evaluating specific molecular sub-types of TNBC that we believe are particularly enhanced in cancer stem cells as potential targets for our product candidates. For example, claudin-low TNBC patients have tumors containing a low level of protein biomarkers called claudins. Claudin-low tumors are highly aggressive and are resistant to treatment. The prognosis for patients with claudin-low TNBC is poor.

Current treatment of breast cancer

Surgery, radiation therapy, targeted therapy, hormone therapy and combinations of conventional chemotherapy are often used to treat breast cancer. However, these therapies carry significant side effects and frequently do not result in a durable clinical response, especially for patients

with TNBC.

The choice of cancer drugs used to treat breast cancer is guided by clinical classification of the tumor as ER positive or negative, PR positive or negative and HER2 positive or negative. The presence, absence or combination of these biomarkers in patient tumors informs the selection of prescribed drugs, which include the anti-estrogen therapies Tamoxifen and aromatase inhibitors, as well as agents that directly target HER2, such as Herceptin and Tykerb. These treatments may slow or stop cancer growth and are currently considered the most successful treatments for breast cancer. However, because TNBC patients are negative for ER, PR and HER2, the treatment options for these patients are limited. In particular, the targeted therapies, including Herceptin, Tykerb and anti-estrogen treatments, are not effective for these patients.

Combinations of conventional chemotherapy work by stopping the function of cancer cells through a variety of mechanisms. Chemotherapies are usually not targeted at any specific differences between cancer cells and

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normal cells. Rather, they kill cancer cells because cancer cells generally grow more rapidly than normal cells and, as a result, are relatively more affected by the chemotherapy than normal cells. Because CSCs exhibit mechanisms of resistance, including a slower rate of growth than other cancer cells, they are often not susceptible to conventional chemotherapy. As a result, the treatments may succeed at initially decreasing tumor burden but ultimately fail to kill the CSCs. For example, in a study conducted at Baylor College of Medicine, in which biopsies were taken from breast cancer patients both before and after conventional chemotherapy treatment, the percentage of CSCs increased over the 12-week treatment period, indicating the survival of these cells.

If tumors recur, which happens more often in TNBC than other breast cancers, further therapy with conventional chemotherapy is generally palliative, not curative, as the CSCs are able to metastasize and spread to other sites in the body.

VS-507

Overview

We are currently evaluating VS-507 in preclinical studies as a potential therapy for breast cancer. Our scientific co-founders identified VS-507 using the proprietary technology that we license from the Whitehead Institute and published the results in the peer reviewed scientific journal *Cell* in 2009. We hold an exclusive license from the Whitehead Institute for use of VS-507 in treating cancer. We expect to initiate a clinical trial with VS-507 over the next 12 to 15 months.

We believe VS-507 targets CSCs by disrupting signaling inside these cells. A group of scientific researchers recently reported in the *Proceedings of the National Academy of Sciences of the United States of America*, or *PNAS*, that VS-507 s activity may be mediated through the blockade of the Wnt/beta-catenin cell signaling pathway. Numerous research reports, including a 2011 paper published in *Cell* by our scientific co-founder Robert Weinberg, describe a critical role of the Wnt/beta-catenin signaling pathway in the development and maintenance of CSCs.

Wnts are a family of proteins that bind to receptor proteins, called Frizzled receptors, on the tumor cell surface. We believe that blocking Wnt function could dramatically impair survival and growth of CSCs. However, Wnt signaling is extremely complex, involving 19 different Wnt proteins stimulating through 10 different Frizzled receptors. While it may be possible to develop a small molecule or antibody that can block binding of one or perhaps a few Wnts to their receptors, such a drug likely would not effectively eliminate CSCs because other Wnt and Frizzled proteins that remain unblocked would be sufficient to maintain CSC function.

A potential breakthrough solution to this problem has come through the identification of the LRP6 protein, which interacts with multiple Wnt proteins and appears to be necessary for the development and maintenance of CSCs. LRP6 may represent a single common point of the Wnt system that can be targeted to kill CSCs. In the *PNAS* study referenced above, VS-507 decreased the levels of LRP6 protein *in vitro* and blocked the ability of Wnt proteins to stimulate beta-catenin, a signaling protein that regulates genes responsible for CSC function. We believe this disruption of the Wnt/beta-catenin signaling pathway is responsible for the inhibitory effects of VS-507 on CSCs that we have observed in preclinical studies.

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Preclinical development

We are conducting a comprehensive preclinical program to study VS-507 as a potential treatment for breast cancer. Key results of this program to date, based on experiments conducted by our scientific co-founders, are summarized below.

Laboratory studies. The effect of VS-507 on CSCs as compared to other cancer cells was evaluated *in vitro*. We believe that a biomarker useful for identifying breast CSCs is the expression ratio of the cell surface proteins CD44 to CD24, which can be measured for each individual cell using a method known as flow cytometry. Using this method, the amount of each protein is measured on the cell surface and the number of CSCs in a cell culture is determined by quantifying cell populations based on their expression of CD44 and CD24. As originally reported in *PNAS* in 2003, breast CSCs express high levels of CD44 and low levels of CD24 relative to other types of breast cancer cells. This differential expression is represented in the figure below as an increase in the shading in the top left portion of the flow cytometry plot. Treatment of a breast cancer cell line containing CSCs with VS-507 resulted in a decrease in the population of CSCs compared to the placebo control. In contrast, treatment with paclitaxel resulted in an increase in the population of CSCs compared to the placebo control. We believe that the opposing actions of VS-507 and paclitaxel are due to a selective effect of VS-507 on the killing of CSCs not observed with paclitaxel treatment.

Gene expression analysis. Opposing effects of VS-507 and paclitaxel also were shown by gene expression analysis. Human breast cancer cells were treated in culture with either VS-507 or paclitaxel for one week and then incubated in the absence of drug for three weeks prior to analysis. The two populations were subjected to comparative global gene expression analysis, which can identify the genes that have the greatest differential change in expression in response to treatment. The panel of genes exhibiting the greatest differential change in this analysis comprise a gene expression signature that may be used for the identification of CSCs. In this experiment, VS-507 and paclitaxel had opposing actions on biomarkers of CSCs and genes known to be commonly expressed in epithelial tissue types. Unlike treatment with paclitaxel, treatment with VS-507 resulted in the loss of expression of CSC-associated genes. Expression of these genes is correlated with poor-prognosis tumors.

Mouse models of breast cancer. The functional presence of CSCs was assessed by evaluating *in vivo* tumor-initiating, or tumor-forming, ability after chemical compound treatment. In these experiments, a human breast cancer cell line containing a mixture of CSCs and other cancer cells was treated with VS-507, paclitaxel or a placebo control *in vitro* for seven days and expanded in culture for at least 14 days in the absence of treatment. The cells were then injected into mice. As shown in the figure below, treatment of these cells with VS-507 resulted in the formation of tumors in fewer mice than treatment with paclitaxel. These findings suggest that CSCs within breast cancer cell populations may be resistant to paclitaxel but sensitive to treatment with VS-507.

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Mouse model of metastatic breast cancer. To specifically evaluate the effects of a therapeutic compound on the metastatic potential of cells following treatment, murine breast cancer cells treated *in vitro* with VS-507, paclitaxel or a placebo control were injected into the tail vein of mice and the number of metastases that subsequently appeared in the lungs was measured. After three weeks of growth of these cells *in vivo*, mice injected with cells that had been treated with VS-507 displayed a four-fold reduction in metastatic burden compared to the placebo control while, in contrast, mice injected with cells that had been treated with paclitaxel displayed a two-fold increase in metastatic burden compared to the placebo control.

VS-507 clinical development plan

Assuming successful completion of preclinical studies, we expect to initiate a clinical trial with VS-507 over the next 12 to 15 months. In a Phase 1 clinical trial, we anticipate enrolling patients with advanced solid tumors. The dose escalation portion of the Phase 1 clinical trial would be designed to determine the maximum tolerated dose of VS-507. We also plan to assess safety and tolerability of VS-507 in this portion of the trial.

Upon identification of the maximum tolerated dose, we plan to enroll an expanded cohort of breast cancer patients to further assess the safety of VS-507 and evaluate efficacy on a preliminary basis in accordance with Response Criteria in Solid Tumors, or RECIST, measurement guidelines, and based on the presence of CSC-specific biomarkers. RECIST has traditionally been used as a standard measure of activity in clinical trials. However, because RECIST is based on gross changes in the size of tumor lesions of more than 30%, it is possible that changes in the tumor burden following selective targeting of CSCs in a single-agent, maximum-tolerated-dose study will not be detected using RECIST. As a result, we believe that sensitive CSC-specific biomarkers may be useful in conjunction with RECIST to quantify the effect of VS-507 on CSCs.

VS-4718 / VS-5095

Overview

We are currently evaluating VS-4718 and VS-5095 as potential therapies for cancers with a high percentage of CSCs. We identified the CSC-targeted activity of these compounds using our proprietary technology and hold worldwide exclusive rights to these compounds and their use. We expect to initiate a clinical trial with one of either VS-4718 or VS-5095 over the next 12 to 15 months.

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We believe VS-4718 and VS-5095 target CSCs through inhibition of FAK signaling. FAK expression is greater in many tumor types compared to normal tissue, particularly in cancers that have a high invasive and metastatic capability. The contact between epithelial cancer cells and connective tissue stimulates FAK signaling. However, epithelial cancer cells that undergo EMT acquire the ability to survive in the absence of contact with connective tissue. We believe that FAK signaling in CSCs may be maintained through alternative mechanisms, thus providing CSCs the ability to survive in the absence of cell contact. Accordingly, we believe that FAK signaling may be a central component of CSC biology that allows CSCs to survive after exiting from a tumor mass and enable metastasis to other sites in the body.

In 2009, our scientific co-founder Robert Weinberg reported in *PNAS* that in a mouse model of breast cancer FAK signaling was required to enable lung metastasis. Epithelial cells, which lack the ability to increase their FAK signaling activity through alternative mechanisms, remained non-metastatic in this model and did not survive dissemination to the lungs. In addition, researchers at McGill University reported in *PNAS* that in a genetically modified mouse model the specific deletion of FAK from the mammary cells prevented primary tumor formation and metastasis.

Scientific research suggests that increased FAK expression and activity is associated with metastatic progression and poor prognosis in multiple cancer types. For example, a 2009 retrospective study published in the *Journal of Clinical Investigation* identified the amplification, or increase in number, of the gene encoding FAK in a large percentage of breast cancers. This gene amplification, and resulting high FAK expression, significantly correlated with the progression of early stage, primary breast cancer to advanced metastatic disease. In an analysis of 295 breast cancer patients that was part of this study, elevated FAK expression was a marker of poor survival. The correlation of elevated FAK expression with poor survival was more significant than and independent of other commonly used clinical parameters, such as hormone receptor status. We believe targeted disruption of the FAK signaling pathway with VS-4718 or VS-5095 may reduce both the primary tumor burden and the ability of CSCs to form metastases.

Preclinical development

We are conducting a preclinical program to study VS-4718 and VS-5095 as potential treatments for breast and other cancers associated with increased FAK activity. Key results to date from preclinical studies of VS-4718 performed by our licensor are summarized below. Comparable studies conducted to date of VS-5095 generally have provided similar overall results as the VS-4718 results.

Biochemical and cellular tests. In biochemical testing, VS-4718 inhibited purified FAK and demonstrated *in vitro* selectivity against a panel of 107 different protein kinases. In addition, in various *in vitro* assessments of cell proliferation using our EMT technology, VS-4718 exhibited potent activity and up to a 25-fold preferential effect, or selectivity, for CSCs as compared to other types of cancer cells.

Pharmacokinetics and tolerability in mice. VS-4718 was well tolerated in mice after both acute and chronic dosing. VS-4718 also exhibited acceptable pharmacokinetics in mice. Pharmacokinetics is the process by which a drug is absorbed, distributed and metabolized in the body. In mouse models assessing pharmacodynamics, a single dose of VS-4718 inhibited FAK activity in tumors over a 12-hour period. Pharmacodynamics refers to the biochemical and physiological effect of a drug on the body.

Mouse models of breast cancer. VS-4718 has exhibited tumor growth inhibition and reduction of metastatic burden in several mouse models of breast cancer. In one experiment, VS-4718 was tested in a model in which TNBC cells were implanted into a mouse and the tumor was allowed to develop. Upon tumor formation, the

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mice were treated with VS-4718 in drinking water at a concentration of 0.5 mg/ml or a placebo control beginning at day 12 through the end of the experiment. As shown in the figure below, the tumor volume in the VS-4718 treatment group was significantly smaller than in the placebo group from day 27 through the end of the experiment. In addition, at day 70 the weight of the primary tumor and the number of lung metastases in the VS-4718 treatment group were both significantly less than in the placebo group.

The vertical line on each data point in the tumor volume figure above represents the standard deviation from the mean. The box and vertical line for each data point in the tumor weight and metastases figures above show the distribution of the data. The square data point inside the box represents the mean. The bottom of the box represents the 25th percentile, the middle line in the box represents the median and the top of the box represents the 75th percentile. The vertical lines projecting from the bottom and top of the box represent the 5th and 95th percentiles.

VS-4718 / VS-5095 development plan

We are progressing both VS-4718 and VS-5095 through additional preclinical efficacy and toxicology studies. It is our intention to select only one of these compounds for clinical development. Upon selection of the lead candidate and assuming successful completion of preclinical studies, we expect to initiate clinical trials with one of either VS-4718 or VS-5095 over the next 12 to 15 months. In a Phase 1 clinical trial, we anticipate enrolling patients with advanced solid tumors. The dose escalation portion of the Phase 1 clinical trial would be designed to determine the maximum tolerated dose. We also plan to assess safety and tolerability in this portion of the trial.

Upon identification of the maximum tolerated dose, we plan to enroll an expanded cohort of patients with breast and other cancers associated with increased FAK activity to further assess the safety of the product candidate and evaluate efficacy on a preliminary basis in accordance with RECIST measurement guidelines, and based on the presence of CSC-specific biomarkers. As with VS-507, it is possible that changes in the tumor burden following selective targeting of CSCs in a single-agent, maximum-tolerated-dose study will not be detected

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using RECIST. As a result, we believe that sensitive CSC-specific biomarkers may be useful in conjunction with RECIST to quantify the effect on CSCs following treatment.

NEW CHEMICAL ENTITIES (NCEs)

We have initiated NCE programs on more than 10 series of chemical compounds identified using our proprietary EMT technology along with high-throughput screening methods. In addition, we have synthesized several drug candidates that are chemically similar to VS-507 and are currently optimizing their activity to block Wnt/beta-catenin signaling and induce selective killing of CSCs.

We evaluate the activity of chemical compounds *in vitro* by measuring their potency and selectivity against CSCs. In general, the more potent a drug is, the lower the dose required for a therapeutic effect. In an *in vitro* assessment of cell proliferation, one of the series of NCE compounds that we have identified has exhibited potent activity and greater than 10-fold selectivity for CSCs as compared to other types of cancer cells. A second series of compounds has shown potent activity and greater than 50-fold selectivity for killing of CSCs compared to its effects on other types of cancer cells. Compounds from our NCE programs also have demonstrated preclinical activity in a broad range of cancer cells, including breast cancer cell lines derived from TNBC tumors in which a high percentage of CSCs have been identified. We are currently evaluating additional proprietary product candidates from our NCE programs in preclinical studies for their use in breast and other cancers.

INTELLECTUAL PROPERTY

We aggressively strive to protect the proprietary technology that we believe is important to our business, including seeking and maintaining patents intended to cover our products and compositions, their methods of use and processes for their manufacture, as well as our diagnostic, biomarker, patient selection and drug discovery technologies and any other inventions that are commercially important to the development of our business. We also rely on trade secrets to protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection.

Our success will depend on our ability to obtain and maintain patent and other proprietary protection for commercially important technology, inventions and know-how related to our business, defend and enforce our patents, preserve the confidentiality of our trade secrets and operate without infringing the valid and enforceable patents and proprietary rights of third parties. We also rely on know-how, continuing technological innovation and in-licensing opportunities to develop and maintain our proprietary position. We seek to obtain domestic and international patent protection, and endeavor to promptly file patent applications for new commercially valuable inventions.

We license a portfolio of patent applications owned by the Whitehead Institute, Harvard and MIT. As of March 15, 2012, we hold exclusive licenses from the Whitehead Institute to three pending U.S. patent applications and one issued U.S. patent, as well as foreign counterparts to these patent applications, and one application under a Patent Cooperation Treaty, or PCT application.

One family of applications licensed from the Whitehead Institute under our drug discovery platform license agreement includes claims covering: methods of identifying compounds that inhibit the growth or survival of CSCs, methods of identifying CSCs and methods of treating cancer,

including methods of selecting courses of treatment for cancer therapy based, for example, on the presence of a biomarker. The application also includes claims to methods of using certain compounds, identified for example by the claimed screening technology, in

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the treatment of cancer. Any U.S. or EU patents that may issue from this application would have a statutory expiration date in 2029.

We also license two families of patent applications from the Whitehead Institute under our cancer diagnostic license agreement that include claims covering: additional methods of identifying CSCs, *in vitro* methods of creating CSCs, for example through activation of the EMT process, progenitor cells and uses for those cells, methods of determining the metastatic potential of a tumor and methods of diagnosing, preventing and treating cancer metastasis. Any U.S. patents that may issue from these applications would have a statutory expiration date in 2025 or 2026. One U.S. patent under this agreement has issued, which includes claims covering certain methods of predicting the likelihood that a tumor will metastasize. Although the statutory expiration date for this patent is in March 2025, the patent is entitled to an additional term under U.S. patent adjustment provisions that expires in December 2028.

We also license from the Whitehead Institute under our cancer diagnostic license agreement a PCT application that includes claims covering compositions, such as cell cultures, that include compounds that can induce epithelial cells to undergo an EMT process, methods of inducing epithelial cells to undergo an EMT process and methods of preparing progenitor cells from epithelial cells. Any U.S. patents that may issue from U.S. national stage applications claiming priority from this PCT application would have a statutory expiration date in 2031.

We have an agreement with the Broad Institute, which grants us under certain circumstances the first right to negotiate a license for intellectual property. This intellectual property includes patent applications and patents covering the use of biomarkers related to the EMT process. This intellectual property also includes compounds that can be used for treatment of cancer. An example is a compound that is identified by screening the effects of compounds on CSCs, notably CSCs created through the EMT process. We exercised this right in February 2012 and entered into a license agreement with the Broad Institute for one patent application.

We also exclusively license a portfolio of patent applications relating to FAK inhibitors from Poniard Pharmaceuticals, Inc., or Poniard. As of March 15, 2012, we hold licenses from Poniard to four patent applications, as well as foreign counterparts to these patent applications. One of these patent applications is owned by The Scripps Research Institute, or Scripps, and licensed to Poniard and the other three are owned by Poniard. The patent application owned by Scripps includes claims covering the composition of matter of compounds, which, for example, can inhibit FAK, and methods of using these compounds to treat disorders such as cancer. Any U.S. or EU patents that may issue from this application would have a statutory expiration date in 2028. The patent applications owned by Poniard include claims covering oral formulations of kinase inhibitors, such as FAK inhibitors, and methods of use thereof, methods of synthesis of certain compounds, for example, certain FAK inhibitors, and methods of use thereof, and methods of using a compound to promote apoptosis in tumor cells. Any U.S. or EU patents that may issue from these applications would have a statutory expiration date in 2030 or 2031.

We have filed and own one patent application directed to formulations of VS-507 and one patent application directed to analogues of VS-507. Any U.S. or EU patents that may issue from these applications would have a statutory expiration date in 2032 or 2033.

We may rely, in some circumstances, on trade secrets to protect our technology. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, in part, by confidentiality agreements with our employees, consultants, scientific advisors and contractors. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have

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adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors. To the extent that our consultants, contractors or collaborators use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

LICENSES

Whitehead Institute for Biomedical Research

Drug discovery platform license agreement

In October 2010, we entered into an exclusive license agreement with the Whitehead Institute, or the drug discovery platform license agreement, which we amended and restated in January 2012, both on its own behalf and as sole and exclusive agent of Harvard and MIT. Under the drug discovery platform license agreement, we acquired an exclusive, royalty-bearing, worldwide license under patent rights owned by the Whitehead Institute, Harvard and MIT to develop, make, use and sell products covered by the licensed patent rights, including VS-507 for use in treating cancer, and to develop and perform licensed processes, in each case, for all human therapeutic, prognostic and diagnostic uses. These exclusive licensed patent rights are described in more detail above under Intellectual Property.

We are required to use commercially reasonable efforts to develop and commercialize licensed products under the agreement. In particular, we are required to fulfill specific development and regulatory milestones by particular dates and, during each calendar year, either spend a specified amount for research and development, actively conduct one or more clinical trials for a licensed product or a product identified using a licensed process that does not constitute a licensed product, which we refer to as an identified product, prepare, file or pursue a filed application for regulatory approval of a licensed product or an identified product, or launch or sell a licensed product or identified product.

Under the agreement, we paid the Whitehead Institute an upfront license fee and reimbursed patent related fees and costs incurred by the Whitehead Institute, Harvard and MIT totaling $104,000 in the aggregate and issued 166,664 shares of our common stock to the Whitehead Institute and entities and individuals affiliated with the Whitehead Institute.

We also agreed to pay the Whitehead Institute annual license maintenance fees, milestone payments, royalties as a percentage of net sales and a percentage of sublicense income that we receive. Annual license maintenance fees are creditable against royalties, which are described below, earned during the same calendar year. Milestone payments are triggered upon the achievement of specified development, regulatory and commercialization milestones and are not creditable against the royalties described below. For each licensed product, we agreed to make milestone payments of up to an aggregate of $1,560,000 plus an additional amount for each subsequent approval of additional indications for a maximum number of licensed products. For each identified product that is not a licensed product, we agreed to make milestone payments of up to an aggregate of $815,000 plus an additional amount for each subsequent approval of additional indications for a maximum number of identified products. Each type of specified milestone payment is payable only for each of the maximum number of licensed products and the maximum number of identified products, as the case may be, to achieve the applicable milestone. In addition, a separate milestone payment is due upon the first commercial sale of each licensed product or identified product that is a diagnostic or prognostic test. A single additional milestone payment is due for the first issuance of licensed patent rights in the United States, the United Kingdom, France, Germany, Spain or Italy. The royalty rate is in the low single digits as a percentage of net

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sales for licensed products that are therapeutics, the mid single digits for licensed products that are diagnostics or prognostics and less than one percent for identified products.

The Whitehead Institute, Harvard and MIT retain the right to, and may grant licenses to other academic and non-profit institutions for the right to, practice the licensed patent rights for research, teaching and educational purposes. The Whitehead Institute, Harvard, MIT or any such other institution could seek to license to third parties any intellectual property rights that it discovers using the licensed patent rights while pursuing these purposes. Under the agreement, we have a right, subject to the Whitehead Institute s obligations under third party research funding agreements, to negotiate a license for any compounds identified prior to a specified date in the Whitehead Institute s laboratory run by Dr. Weinberg that selectively target CSCs generated by induction through the EMT process.

After a specified period of time, if a third party requests to sublicense the patent rights for a product or process that is not directly competitive with our products or processes, we must enter into good-faith negotiations to grant a sublicense for such proposed product or process. If we do not grant a sublicense within a specified period of time after receiving a written request, the Whitehead Institute may grant a license to the third party and our rights in the field of use of such sublicense will terminate. Additionally, after a specified period of time, if we are not actively conducting high-throughput screening using the licensed patent rights to identify product candidates, then, except for any rights directed to uses that we are actively developing, the Whitehead Institute may convert our license to the licensed patent rights from exclusive to non-exclusive.

We have the right to terminate the agreement for any reason upon at least 90 days prior written notice. The Whitehead Institute has the right to terminate the agreement if we and all of our sublicensees cease to carry on business related to the agreement for a specified period of time, we fail to pay any amounts due and payable under the agreement to the Whitehead Institute, subject to a grace period, we materially breach the agreement and fail to cure such breach within a specified grace period or we or a sublicensee challenge the licensed patent rights in a legal or administrative proceeding. The agreement otherwise terminates upon the expiration or abandonment of all licensed patents and patent applications.

Cancer diagnostic license agreement

In October 2010, we entered into a separate license agreement with the Whitehead Institute, or the cancer diagnostic license agreement, under which we acquired a non-exclusive, worldwide license to patent rights owned by the Whitehead Institute for research purposes. In December 2011, we amended and restated this agreement with the Whitehead Institute. Under the amended and restated cancer diagnostic license agreement, we acquired an exclusive, royalty-bearing, worldwide license under these patent rights to develop, make, use and sell products covered by the licensed patent rights and to develop and perform services using a licensed product or the practice of the licensed patent rights for or on behalf of a third party, in each case, for cancer diagnostics and companion clinical uses. These licensed patent rights are described in more detail above under Intellectual Property.

Under the agreement, we paid the Whitehead Institute upfront license fees and expect to reimburse patent related fees and costs incurred by the Whitehead Institute totaling $70,000 in the aggregate. We also agreed to pay the Whitehead Institute annual license maintenance fees, milestone payments, royalties as a percentage of net sales and a percentage of sublicense income that we receive. Annual license maintenance fees are creditable against royalties, which are described below, earned during the same calendar year. Milestone payments of up to an aggregate of $825,000 are triggered upon the achievement of specified regulatory and commercialization

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milestones and are not creditable against the royalties described below. The royalty rate is in the mid single digits as a percentage of net sales.

If we are required to pay royalties to a third party in consideration of a license or similar right in order to make, use or sell a licensed product or licensed service, then we may deduct up to 50% of the amounts paid to such third party, subject to specified limitations, from the payments that we owe to the Whitehead Institute for such licensed product or licensed service.

We are required to use commercially reasonable efforts to develop and commercialize licensed products or licensed services under the agreement. In particular, we are required to fulfill specific development, regulatory and commercialization milestones by particular dates and to commit a specified number of full time staff equivalents toward the development of a licensed product or licensed service until the first commercial sale of a licensed product or performance of a licensed service.

The Whitehead Institute retains the right to, and may grant licenses to other academic and non-profit institutions for the right to, practice the licensed patent rights for research, teaching and educational purposes. The Whitehead Institute or any such other institution could seek to license to third parties any intellectual property rights that it discovers using the licensed patent rights while pursuing these purposes.

After a specified period of time, if a third party requests to sublicense the patent rights for a product or service that is not directly competitive with our products or services, we must enter into good-faith negotiations to grant a sublicense for such proposed product or service. If we do not grant such a sublicense within a specified period of time after receiving a written request, the Whitehead Institute may grant a license to the third party and our rights in the field of use of such sublicense will terminate. Additionally, after a specified period of time, if the market is not being reasonably served by us, as determined by the Whitehead Institute, and a third party requests to sublicense the patent rights for a product or service that is directly competitive with our products or services, we must enter into good-faith negotiations to grant a sublicense for such proposed product or service. If we do not grant such a sublicense within a specified period of time after receiving a written request, we and the Whitehead Institute have agreed to mutually select a qualified independent third party to set commercially reasonable terms and conditions consistent with similar technology in the industry under which we would sublicense our rights for such proposed product or service to the third party. Additionally, after a specified period of time, if we are not actively conducting efforts to validate, use or commercialize a license product or licensed service, then the Whitehead Institute may convert our license to the licensed patent rights from exclusive to nonexclusive.

We have the right to terminate the agreement for any reason upon at least 90 days prior written notice. The Whitehead Institute has the right to terminate the agreement if we and all of our sublicensees cease to carry on business related to the agreement for a specified period of time, we fail to pay any amounts due and payable under the agreement to the Whitehead Institute, subject to a grace period, we materially breach the agreement and fail to cure such breach within a specified grace period or we or a sublicensee challenge the licensed patent rights in a legal or administrative proceeding. The agreement otherwise terminates upon the expiration or abandonment of all licensed patents and patent applications.

Broad Institute of MIT and Harvard University

In October 2010, the Broad Institute granted to us the first right to negotiate a license in good faith for specified intellectual property owned by the Broad Institute if we have not breached the terms of the drug discovery platform license agreement described above. Following written notice of the availability of such intellectual

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property for licensing by the Broad Institute to us, the Broad Institute has agreed not to negotiate with any other party during our right of first negotiation period. If we and the Broad Institute are unable to negotiate a license within such period, the Broad Institute may then offer the intellectual property for licensing to other parties. The intellectual property subject to this right of first negotiation is described in more detail above under Intellectual Property.

In February 2012, we entered into an exclusive patent license agreement with the Broad Institute for a patent application covering therapeutic uses of cancer stem cell technology that the Broad Institute offered to us and, in the future, we may enter into one or more additional license agreements with the Broad Institute for cancer stem cell intellectual property if the Broad Institute offers additional licensing opportunities to us pursuant to this right of first negotiation agreement.

Poniard Pharmaceuticals, Inc.

In November 2011, we entered into a license agreement with Poniard under which we acquired an exclusive, worldwide license under patent rights and know-how owned or controlled by Poniard to develop, make, use and sell compounds and products covered by the licensed patent rights for the diagnosis, treatment, prevention or control of all human diseases and conditions. The licensed compounds include VS-4718 and VS-5095 and any other compounds covered by a licensed patent right under the agreement that have the inhibition of FAK as a primary mode of action. These licensed patent rights are described in more detail above under Intellectual Property and include patent rights owned by Scripps and licensed to Poniard. In accordance with the agreement between Poniard and Scripps, Scripps retains the right to grant non-exclusive licenses, without the right to sublicense, to nonprofit or academic institutions to use for any noncommercial research or education purposes any licensed patent rights owned by Scripps and licensed to Poniard.

Under the agreement, we paid Poniard an upfront license fee and agreed to pay Poniard milestone payments of up to an aggregate of $13,250,000 upon the achievement of specified development and regulatory milestones. We also agreed to issue to Poniard a warrant to purchase 142,857 shares of our common stock upon the first dosing of the first patient in our first Phase 1 clinical trial of a licensed product. The exercise price of such warrant would be equal to the average closing price of our common stock during the five trading days preceding such issue date. In addition, we agreed to pay low to mid single digit royalties to Poniard as a percentage of net sales of licensed products. Our obligation to pay royalties continues on a country by country basis until the expiration of all licensed patent rights covering licensed products in such country. If the royalty term under our agreement with Poniard expires with respect to a licensed product in a country and Poniard continues to have royalty payment obligations under its agreement with Scripps with respect to our net sales of licensed products in such country, we agreed to pay Poniard the royalty amount due to Scripps with respect to net sales of such licensed product in such country.

Poniard is responsible for all amounts payable to any third party under any agreement to which Poniard was a party as of the date of our agreement that are applicable to rights licensed to us, including amounts payable to Scripps with respect to the patent rights owned by Scripps and licensed to Poniard. If we license or acquire technology from a third party in order to develop or commercialize a licensed product and are required to pay such third party license fees, milestone payments, royalties or other amounts, then we may deduct up to 50% of the amount paid to such third party from the payments that we owe to Poniard for such licensed product. This deduction is subject to specified limitations, including that in no event will any such deduction reduce a payment that we owe to Poniard to less than 50% of the otherwise applicable amount.

We are required to use commercially reasonable efforts to develop and, subject to regulatory approval, commercialize licensed products in the United States, the United Kingdom, France, Germany and Japan.

We have the right to terminate the agreement or any portion of our licensed rights under the agreement upon at least 90 days prior written notice. We and Poniard each have the right to terminate the agreement if the other party materially breaches the agreement and fails to cure such breach within a specified grace period, subject to

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the right of either party to submit a dispute to arbitration. The agreement otherwise terminates upon the last to expire licensed patent right covering a licensed product.

COMPETITION

The biotechnology and pharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. While we believe that our technology, development experience and scientific knowledge provide us with competitive advantages, we face potential competition from many different sources, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions and governmental agencies and public and private research institutions. Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future.

There are other companies working to develop therapies that target CSCs. These companies include divisions of large pharmaceutical companies including Astellas Pharma Inc., Sanofi-Aventis U.S. 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.

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

The key competitive factors affecting the success of all of our product candidates, if approved, are likely to be their efficacy, safety, convenience, price, the effectiveness of companion diagnostics in guiding the use of related therapeutics, the level of generic competition and the availability of reimbursement from government and other third-party payors.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval 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. In addition, our ability to compete may be affected in many cases by insurers or other third-party payors seeking to encourage the use of generic products. There are many generic products currently on the market for the indications that we are pursuing, and additional products are expected to become available on a generic basis over the coming years. If our therapeutic product candidates are approved, we expect that they will be priced at a significant premium over competitive generic products.

The most common methods of treating patients with cancer are surgery, radiation and drug therapy, including chemotherapy, hormone therapy and targeted drug therapy. There are a variety of available drug therapies marketed for cancer. In many cases, these drugs are administered in combination to enhance efficacy. While our

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product candidates may compete with many existing drug and other therapies, to the extent they are ultimately used in combination with or as an adjunct to these therapies, our product candidates will not be competitive with them. Some of the currently approved drug therapies 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. In general, although there has been considerable progress over the past few decades in the treatment of cancer and the currently marketed therapies provide benefits to many patients, these therapies all are limited to some extent in their efficacy and frequency of adverse events, and none of them are successful in treating all patients. As a result, the level of morbidity and mortality from cancer remains high.

In addition to currently marketed therapies, there are also a number of products in late stage clinical development to treat cancer. These products in development may provide efficacy, safety, convenience and other benefits that are not provided by currently marketed therapies. As a result, they may provide significant competition for any of our product candidates for which we obtain market approval.

MANUFACTURING

We do not own or operate, and currently have no plans to establish, any manufacturing facilities. We currently rely, and expect to continue to rely, on third parties for the manufacture of our product candidates and any products that we may develop, other than small amounts of compounds that we may synthesize ourselves for preclinical testing. To date, we have obtained starting materials for our supply of the bulk drug substance for our product candidates from one third-party manufacturer. We obtain our supplies from this manufacturer on a purchase order basis and do not have a long-term supply arrangement in place. We do not currently have arrangements in place for redundant supply or a second source for bulk drug substance. If our current third-party manufacturer should become unavailable to us for any reason, we believe that there are several potential replacements, although we might incur some delay in identifying and qualifying such replacements.

All of our drug candidates are organic compounds of low molecular weight, generally called small molecules. We select compounds not only on the basis of their potential efficacy and safety, but also for their ease of synthesis and reasonable cost of their starting materials. We expect to continue to develop drug candidates that can be produced cost-effectively at third-party manufacturing facilities.

GOVERNMENT REGULATION

Government authorities in the United States, at the federal, state and local level, and in other countries extensively regulate, among other things, the research, development, testing, manufacture, including any manufacturing changes, packaging, storage, recordkeeping, labeling, advertising, promotion, distribution, marketing, post-approval monitoring and reporting, import and export of pharmaceutical products, such as those we are developing.

United States drug approval process

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, or FDCA, the Public Health Service Act and implementing regulations. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local

and foreign statutes and regulations requires the expenditure of substantial time and financial resources. Failure to comply with the applicable United States requirements at any time during the product development process, approval process or after approval, may subject an applicant to a variety of administrative or judicial sanctions, such as the FDA s refusal to approve

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pending applications, withdrawal of an approval, imposition of a clinical hold, issuance of warning letters, product recalls, product seizures, total or partial suspension of production or distribution injunctions, fines, refusals of government contracts, restitution, disgorgement of profits or civil or criminal penalties.

The process required by the FDA before a drug may be marketed in the United States generally involves the following:

- completion of preclinical laboratory tests, animal studies and formulation studies in compliance with the FDA s good laboratory practice, or GLP, regulations;

- submission to the FDA of an IND, which must become effective before human clinical trials may begin;

- approval by an independent institutional review board, or IRB, at each clinical site before each trial may be initiated;

- performance of adequate and well-controlled human clinical trials in accordance with good clinical practices, or GCP, to establish the safety and efficacy of the proposed drug for each indication;

- submission to the FDA of a new drug application, or NDA;

- satisfactory completion of an FDA advisory committee review, if applicable;

- satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the product is produced to assess compliance with current good manufacturing practices, or cGMP, requirements and to assure that the facilities, methods and controls are adequate to preserve the drug s identity, strength, quality and purity; and

- FDA review and approval of the NDA.

Preclinical studies

Preclinical studies include laboratory evaluation of product chemistry and formulation, as well as *in vitro* and animal studies to assess the potential for adverse events and in some cases to establish a rationale for therapeutic use. The conduct of preclinical studies is subject to federal regulations and requirements, including GLP regulations for safety/toxicology studies. An IND sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and plans for clinical studies, among other things, to the FDA as part of an IND. Some long-term preclinical testing, such as animal tests of reproductive adverse events and carcinogenicity, may continue after the IND is submitted. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to one or more proposed clinical trials and places the trial on clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. As a result, submission of an IND may not result in the FDA allowing clinical trials to commence.

Clinical trials

Clinical trials involve the administration of the investigational new drug to human subjects under the supervision of qualified investigators in accordance with GCP requirements, which include, among other things, the requirement that all research subjects provide their informed consent in writing before their participation in any clinical trial. Clinical trials are conducted under written study protocols detailing, among other things, the

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objectives of the study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND. In addition, an IRB at each institution participating in the clinical trial must review and approve the plan for any clinical trial before it commences at that institution, and the IRB must conduct continuing review. The IRB must review and approve, among other things, the study protocol and informed consent information to be provided to study subjects. An IRB must operate in compliance with FDA regulations. Information about certain clinical trials must be submitted within specific timeframes to the National Institutes of Health for public dissemination on their ClinicalTrials.gov website.

Human clinical trials are typically conducted in three sequential phases, which may overlap or be combined:

- *Phase 1:* The drug is initially introduced into healthy human subjects or patients with the target disease or condition and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early indication of its effectiveness.

- *Phase 2:* The drug is administered to a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage.

- *Phase 3:* The drug is administered to an expanded patient population in adequate and well-controlled clinical trials to generate sufficient data to statistically confirm the efficacy and safety of the product for approval, to establish the overall risk-benefit profile of the product and to provide adequate information for the labeling of the product.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and more frequently if serious adverse events occur. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, or at all. Furthermore, the FDA or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB s requirements or if the drug has been associated with unexpected serious harm to patients.

Marketing approval

Assuming successful completion of the required clinical testing, the results of the preclinical and clinical studies, together with detailed information relating to the product s chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of an NDA requesting approval to market the product for one or more indications. Under federal law, the submission of most NDAs is additionally subject to a substantial application user fee, currently exceeding $1.8 million, and the sponsor of an approved NDA is also subject to annual product and establishment user fees, currently exceeding $98,000 per product and $520,000 per establishment. These fees are typically increased annually.

The FDA conducts a preliminary review of all NDAs within the first 60 days after submission before accepting them for filing to determine whether they are sufficiently complete to permit substantive review. The FDA may request additional information rather than accept an NDA for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review. The FDA has agreed to specified performance goals in the review of NDAs. Under these goals, the

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FDA has committed to review most such applications for non-priority products within 10 months, and most applications for priority review products, that is, drugs that the FDA determines represent a significant improvement over existing therapy, within six months. These performance goals likely will be extended by several months when the Prescription Drug User Fee Act is reauthorized in 2012. The review process may be extended by the FDA for three additional months to consider certain information or clarification regarding information already provided in the submission. The FDA may also refer applications for novel drugs or products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Before approving an NDA, the FDA typically will inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. In addition, before approving an NDA, the FDA will typically inspect one or more clinical sites to assure compliance with GCP and integrity of the clinical data submitted.

The testing and approval process requires substantial time, effort and financial resources, and each may take many years to complete. Data obtained from clinical activities are not always conclusive and may be susceptible to varying interpretations, which could delay, limit or prevent regulatory approval. The FDA may not grant approval on a timely basis, or at all. We may encounter difficulties or unanticipated costs in our efforts to develop our product candidates and secure necessary governmental approvals, which could delay or preclude us from marketing our products.

After the FDA s evaluation of the NDA and inspection of the manufacturing facilities, the FDA may issue an approval letter or a complete response letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. If and when those deficiencies have been addressed to the FDA s satisfaction in a resubmission of the NDA, the FDA will issue an approval letter. The FDA has committed to reviewing such resubmissions in two or six months depending on the type of information included. Even with submission of this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval and refuse to approve the NDA.

Even if the FDA approves a product, it may limit the approved indications for use for the product, require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess a drug s safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution restrictions or other risk management mechanisms, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-market studies or surveillance programs. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further testing requirements and FDA review and approval.

Fast track designation

The FDA is required to facilitate the development and expedite the review of drugs that are intended for the treatment of a serious or life-threatening condition for which there is no effective treatment and which

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demonstrate the potential to address unmet medical needs for the condition. Under the fast track program, the sponsor of a new drug candidate may request the FDA to designate the product for a specific indication as a fast track product concurrent with or after the filing of the IND for the product candidate. The FDA must determine if the product candidate qualifies for fast track designation within 60 days after receipt of the sponsor s request.

In addition to other benefits, such as the ability to use surrogate endpoints and have greater interactions with the FDA, the FDA may initiate review of sections of a fast track product s NDA before the application is complete. This rolling review is available if the applicant provides and the FDA approves a schedule for the submission of the remaining information and the applicant pays applicable user fees. However, the FDA s time period goal for reviewing a fast track application does not begin until the last section of the NDA is submitted. In addition, the fast track designation may be withdrawn by the FDA if the FDA believes that the designation is no longer supported by data emerging in the clinical trial process.

Priority review

Under FDA policies, a product candidate may be eligible for priority review, or review within a six-month time frame from the time a complete application is received. Products regulated by the FDA s Center for Drug Evaluation and Research, or CDER, are eligible for priority review if they provide a significant improvement compared to marketed products in the treatment, diagnosis or prevention of a disease. A fast track designated product candidate would ordinarily meet the FDA s criteria for priority review.

Accelerated approval

Under the FDA s accelerated approval regulations, the FDA may approve a drug for a serious or life-threatening illness that provides meaningful therapeutic benefit to patients over existing treatments based upon a surrogate endpoint that is reasonably likely to predict clinical benefit. In clinical trials, a surrogate endpoint is a measurement of laboratory or clinical signs of a disease or condition that substitutes for a direct measurement of how a patient feels, functions or survives. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. A product candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, would allow the FDA to withdraw the drug from the market on an expedited basis. All promotional materials for drug candidates approved under accelerated regulations are subject to prior review by the FDA.

Orphan drugs

Under the Orphan Drug Act, the FDA may grant orphan drug designation to drugs intended to treat a rare disease or condition, which is generally defined as a disease or condition that affects fewer than 200,000 individuals in the United States. Orphan drug designation must be requested before submitting an NDA. After the FDA grants orphan drug designation, the generic identity of the drug and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. The first NDA applicant to receive FDA approval for a particular active ingredient to treat a particular disease with FDA orphan drug designation is entitled to a seven-year exclusive marketing period in the United States for that product, for that indication. During the seven-year exclusivity period, the FDA may not approve any other applications to market the same drug for the same orphan indication,

except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity in that it is shown to be safer, more effective or makes a major contribution to patient

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care. Orphan drug exclusivity does not prevent the FDA from approving a different drug for the same disease or condition, or the same drug for a different disease or condition. Among the other benefits of orphan drug designation are tax credits for certain research and a waiver of the NDA application user fee.

Pediatric information

Under the Pediatric Research Equity Act of 2003, as amended and reauthorized by the Food and Drug Administration Amendments Act of 2007, or the FDAAA, an NDA or supplement to an NDA must contain data that are adequate to assess the safety and effectiveness of the drug for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. Unless otherwise required by regulation, the pediatric data requirements do not apply to products with orphan drug designation.

The Hatch-Waxman act

Abbreviated new drug applications

In seeking approval for a drug through an NDA, applicants are required to list with the FDA each patent with claims that cover the applicant s product or a method of using the product. Upon approval of a drug, each of the patents listed in the application for the drug is then published in the FDA s Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book. Drugs listed in the Orange Book can, in turn, be cited by potential competitors in support of approval of an abbreviated new drug application, or ANDA. Generally, an ANDA provides for marketing of a drug product that has the same active ingredients in the same strengths, dosage form and route of administration as the listed drug and has been shown to be bioequivalent through *in vitro* or *in vivo* testing or otherwise to the listed drug. ANDA applicants are not required to conduct or submit results of preclinical or clinical tests to prove the safety or effectiveness of their drug product, other than the requirement for bioequivalence testing. Drugs approved in this way are commonly referred to as generic equivalents to the listed drug, and can often be substituted by pharmacists under prescriptions written for the original listed drug.

The ANDA applicant is required to certify to the FDA concerning any patents listed for the approved product in the FDA s Orange Book, except for patents covering methods of use for which the ANDA applicant is not seeking approval. Specifically, the applicant must certify with respect to each patent that:

- the required patent information has not been filed;
- the listed patent has expired;

- the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or

- the listed patent is invalid, unenforceable or will not be infringed by the new product.

A certification that the new product will not infringe the already approved product s listed patents or that such patents are invalid or unenforceable is called a Paragraph IV certification. If the applicant does not challenge the listed patents or indicate that it is not seeking approval of a patented method of use, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired.

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If the ANDA applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders once the ANDA has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days after the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months, expiration of the patent, settlement of the lawsuit or a decision in the infringement case that is favorable to the ANDA applicant.

The ANDA also will not be approved until any applicable non-patent exclusivity period, such as exclusivity for obtaining approval of a new chemical entity, for the referenced product has expired. Federal law provides a period of five years following approval of a drug containing no previously approved active moiety during which ANDAs for generic versions of those drugs cannot be submitted unless the submission contains a Paragraph IV challenge to a listed patent, in which case the submission may be made four years following the original product approval. Federal law provides for a period of three years of exclusivity during which the FDA cannot grant effective approval of an ANDA if a listed drug contains a previously approved active moiety, but FDA requires as a condition of approval new clinical trials conducted by or for the sponsor. This three-year exclusivity period often protects changes to a previously approved drug product, such as a new dosage form, route of administration, combination or indication. Under the Best Pharmaceuticals for Children Act, federal law also provides that periods of patent and non-patent marketing exclusivity listed in the Orange Book for a drug may be extended by six months if the NDA sponsor conducts pediatric studies identified by the FDA in a written request. For written requests issued by the FDA after September 27, 2007, the date of enactment of the FDAAA, the FDA must grant pediatric exclusivity no later than nine months prior to the date of expiration of patent or non-patent exclusivity in order for the six-month pediatric extension to apply to that exclusivity period.

Section 505(b)(2) new drug applications

Most drug products obtain FDA marketing approval pursuant to an NDA or an ANDA. A third alternative is a special type of NDA, commonly referred to as a Section 505(b)(2) NDA, which enables the applicant to rely, in part, on the FDA s previous approval of a similar product, or published literature, in support of its application.

505(b)(2) NDAs often provide an alternate path to FDA approval for new or improved formulations or new uses of previously approved products. Section 505(b)(2) permits the filing of an NDA where at least some of the information required for approval comes from studies not conducted by or for the applicant and for which the applicant has not obtained a right of reference. If the 505(b)(2) applicant can establish that reliance on the FDA s previous approval is scientifically appropriate, it may eliminate the need to conduct certain preclinical or clinical studies of the new product. The FDA may also require companies to perform additional studies or measurements to support the change from the approved product. The FDA may then approve the new product candidate for all or some of the label indications for which the referenced product has been approved, as well as for any new indication sought by the Section 505(b)(2) applicant.

To the extent that the Section 505(b)(2) applicant is relying on studies conducted for an already approved product, the applicant is required to certify to the FDA concerning any patents listed for the approved product in the Orange Book to the same extent that an ANDA applicant would. As a result, approval of a 505(b)(2) NDA can be stalled until all the listed patents claiming the referenced product have expired, until any non-patent exclusivity, such as exclusivity for obtaining approval of a new chemical entity, listed in the Orange Book for the referenced product has expired, and, in the case of a Paragraph IV certification and subsequent patent

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infringement suit, until the earlier of 30 months, settlement of the lawsuit or a decision in the infringement case that is favorable to the Section 505(b)(2) applicant.

Combination products

The FDA regulates combinations of products that cross FDA centers, such as drug, biologic or medical device components that are physically, chemically or otherwise combined into a single entity, as a combination product. The FDA center with primary jurisdiction for the combination product will take the lead in the premarket review of the product, with the other center consulting or collaborating with the lead center.

The FDA s Office of Combination Products, or OCP, determines which center will have primary jurisdiction for the combination product based on the combination product s primary mode of action. A mode of action is the means by which a product achieves an intended therapeutic effect or action. The primary mode of action is the mode of action that provides the most important therapeutic action of the combination product, or the mode of action expected to make the greatest contribution to the overall intended therapeutic effects of the combination product.

Often it is difficult for the OCP to determine with reasonable certainty the most important therapeutic action of the combination product. In those difficult cases, the OCP will consider consistency with other combination products raising similar types of safety and effectiveness questions, or which center has the most expertise to evaluate the most significant safety and effectiveness questions raised by the combination product.

A sponsor may use a voluntary formal process, known as a Request for Designation, when the product classification is unclear or in dispute, to obtain a binding decision as to which center will regulate the combination product. If the sponsor objects to that decision, it may request that the agency reconsider that decision.

Overview of FDA regulation of companion diagnostics

We are developing *in vitro* and *in vivo* companion diagnostics for use in selecting the patients that we believe will respond to our cancer therapeutics.

FDA officials have issued draft guidance that, when finalized, would address issues critical to developing *in vitro* companion diagnostics, such as biomarker qualification, establishing clinical validity, the use of retrospective data, the appropriate patient population and when the FDA will require that the device and the drug be approved simultaneously. The draft guidance issued in July 2011 states that if safe and effective use of a therapeutic product depends on an *in vitro* diagnostic, then the FDA generally will require approval or clearance of the diagnostic at the same time that the FDA approves the therapeutic product. The FDA has yet to issue further guidance, and it is unclear whether it will do so, or what the scope would be.

The FDA previously has required *in vitro* companion diagnostics intended to select the patients who will respond to the cancer treatment to obtain Pre-Market Approval, or PMA, simultaneously with approval of the drug. Based on the draft guidance, and the FDA s past treatment of companion diagnostics, we believe that the FDA will require one or more of our *in vitro* companion diagnostics to obtain PMA for our companion diagnostics to identify patient populations suitable for our cancer therapies, such as the *in vitro* companion diagnostic for VS-507, VS-4818 or VS-5095. The review of these *in vitro* companion diagnostics in conjunction with the review of our cancer treatments involves coordination of review by CDER and by the FDA s Center for Devices and Radiological Health Office of In Vitro Diagnostics Device Evaluation and Safety.

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PMA approval pathway

A medical device, including an *in vitro* diagnostic, or IVD, to be commercially distributed in the United States must receive either 510(k) clearance or PMA approval from the FDA prior to marketing. Devices deemed by the FDA to pose the greatest risk, such as life-sustaining, life supporting or implantable devices, or devices deemed not substantially equivalent to a previously 510(k) cleared device or a preamendment class III device for which PMA applications have not been called, are placed in Class III requiring PMA approval. The PMA approval pathway requires proof of the safety and effectiveness of the device to the FDA s satisfaction.

The PMA approval pathway generally takes from one to three years or even longer from submission of the application.

A PMA application for an IVD must provide extensive preclinical and clinical trial data. Preclinical data for an IVD includes many different tests, including how reproducible the results are when the same sample is tested multiple times by multiple users at multiple laboratories. The clinical data need to establish that the test is sufficiently safe, effective and reliable in the intended use population. In addition, the FDA must be convinced that a device has clinical utility, meaning that an IVD provides information that is clinically meaningful. A biomarker s clinical significance may be obvious, or the applicant may be able to rely upon published literature or submit data to show clinical utility.

A PMA application also must provide information about the device and its components regarding, among other things, device design, manufacturing and labeling. The sponsor must pay an application fee.

As part of the PMA review, the FDA will typically inspect the manufacturer s facilities for compliance with Quality System Regulation, or QSR, requirements, which impose elaborate testing, control, documentation and other quality assurance procedures.

Upon submission, the FDA determines if the PMA application is sufficiently complete to permit a substantive review, and, if so, the FDA accepts the application for filing. The FDA then commences an in-depth review of the PMA application. The entire process typically takes one to three years, but may take longer. The review time is often significantly extended as a result of the FDA asking for more information or clarification of information already provided. The FDA also may respond with a not approvable determination based on deficiencies in the application and require additional clinical trials that are often expensive and time-consuming and can substantially delay approval.

During the review period, an FDA advisory committee, typically a panel of clinicians, may be convened to review the application and recommend to the FDA whether, or upon what conditions, the device should be approved. Although the FDA is not bound by the advisory panel decision, the panel s recommendation is important to the FDA s overall decision making process.

If the FDA s evaluation of the PMA application is favorable, the FDA typically issues an approvable letter requiring the applicant s agreement to specific conditions, such as changes in labeling, or specific additional information, such as submission of final labeling, in order to secure final approval of the PMA. If the FDA concludes that the applicable criteria have been met, the FDA will issue a PMA for the approved indications, which can be more limited than those originally sought by the manufacturer. The PMA can include post-approval conditions that the FDA believes necessary to ensure the safety and effectiveness of the device, including, among other things, restrictions on labeling, promotion, sale

and distribution. Failure to comply with

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the conditions of approval can result in material adverse enforcement action, including the loss or withdrawal of the approval.

Even after approval of a PMA, a new PMA or PMA supplement may be required in the event of a modification to the device, its labeling or its manufacturing process. Supplements to a PMA often require the submission of the same type of information required for an original PMA, except that the supplement is generally limited to the information needed to support the proposed change from the product covered by the original PMA.

Clinical trials

A clinical trial is almost always required to support a PMA application. In some cases, one or more smaller Investigational Device Exemption, or IDE, studies may precede a pivotal clinical trial intended to demonstrate the safety and efficacy of the investigational device.

All clinical studies of investigational devices must be conducted in compliance with the FDA s requirements. If an investigational device could pose a significant risk to patients pursuant to FDA regulations, the FDA must approve an IDE application prior to initiation of investigational use. IVD trials usually do not require an IDE, as the FDA does not judge them to be a significant risk because the results do not affect the patients in the study. However, for a trial where the IVD result directs the therapeutic care of patients with cancer, we believe that the FDA would consider the investigation to present significant risk.

An IDE application must be supported by appropriate data, such as laboratory test results, showing that it is safe to test the device in humans and that the testing protocol is scientifically sound. The FDA typically grants IDE approval for a specified number of patients. A nonsignificant risk device does not require FDA approval of an IDE. Both significant risk and nonsignificant risk investigational devices require approval from IRBs at the study centers where the device will be used.

During the trial, the sponsor must comply with the FDA s IDE requirements for investigator selection, trial monitoring, reporting and record keeping. The investigators must obtain patient informed consent, rigorously follow the investigational plan and study protocol, control the disposition of investigational devices and comply with all reporting and record keeping requirements. Prior to granting PMA approval, the FDA typically inspects the records relating to the conduct of the study and the clinical data supporting the PMA application for compliance with applicable requirements.

Although the QSR does not fully apply to investigational devices, the requirement for controls on design and development does apply. The sponsor also must manufacture the investigational device in conformity with the quality controls described in the IDE application and any conditions of IDE approval that the FDA may impose with respect to manufacturing.

Post-market

After a device is on the market, numerous regulatory requirements apply. These requirements include: the QSR, labeling regulations, the FDA s general prohibition against promoting products for unapproved or off label uses, the Medical Device Reporting regulation, which requires that manufacturers report to the FDA if their device may have caused or contributed to a death or serious injury or malfunctioned in a way that would likely cause or contribute to a death or serious injury if it were to recur, and the Reports of Corrections and Removals regulation, which requires manufacturers to report recalls and field actions to the FDA if initiated to reduce a risk to health posed by the device or to remedy a violation of the FDCA.

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The FDA enforces these requirements by inspection and market surveillance. If the FDA finds a violation, it can institute a wide variety of enforcement actions, ranging from a public warning letter to more severe sanctions such as: fines, injunctions and civil penalties; recall or seizure of products; operating restrictions, partial suspension or total shutdown of production; refusing requests for PMA approval of new products; withdrawing PMA approvals already granted; and criminal prosecution.

Other regulatory requirements

Any drug manufactured or distributed by us pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion and reporting of adverse experiences with the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims are subject to prior FDA review and approval.

The FDA may impose a number of post-approval requirements as a condition of approval of an NDA. For example, the FDA may require post-marketing testing, including Phase 4 clinical trials, and surveillance to further assess and monitor the product s safety and effectiveness after commercialization. Regulatory approval of oncology products often requires that patients in clinical trials be followed for long periods to determine the overall survival benefit of the drug.

In addition, drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and state agencies, and are subject to periodic unannounced inspections by the FDA and these state agencies for compliance with cGMP requirements. Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon us and any third-party manufacturers that we may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the areas of production and quality control to maintain cGMP compliance.

Once an approval is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information, imposition of post-market studies or clinical trials to assess new safety risks or imposition of distribution or other restrictions under a Risk Evaluation and Mitigation Strategy program. Other potential consequences include, among other things:

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

- fines, warning letters or holds on post-approval clinical trials;

- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of product license approvals;

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

- consent decrees, injunctions or the imposition of civil or criminal penalties.

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The FDA strictly regulates marketing, labeling, advertising and promotion of products that are placed on the market. Drugs may be promoted only for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off label uses, and a company that is found to have improperly promoted off label uses may be subject to significant liability.

Additional provisions

Anti-kickback and false claims laws

In addition to FDA restrictions on marketing of pharmaceutical products, several other types of state and federal laws have been applied to restrict certain marketing practices in the pharmaceutical industry in recent years. These laws include anti-kickback statutes and false claims statutes. The federal healthcare program anti-kickback statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any healthcare item or service reimbursable under Medicare, Medicaid or other federally financed healthcare programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers and formulary managers on the other. Violations of the anti-kickback statute are punishable by imprisonment, criminal fines, civil monetary penalties and exclusion from participation in federal healthcare programs. Although there are a number of statutory exemptions and regulatory safe harbors protecting certain common activities from prosecution or other regulatory sanctions, the exemptions and safe harbors are drawn narrowly, and practices that involve remuneration intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor.

Federal false claims laws prohibit any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to have a false claim paid. Recently, several pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly inflating drug prices they report to pricing services, which in turn were used by the government to set Medicare and Medicaid reimbursement rates, and for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. In addition, certain marketing practices, including off-label promotion, may also violate false claims laws. The majority of states also have statutes or regulations similar to the federal anti-kickback law and false claims laws, which apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor.

Physician drug samples

As part of the sales and marketing process, pharmaceutical companies frequently provide samples of approved drugs to physicians. The Prescription Drug Marketing Act, or the PDMA, imposes requirements and limitations upon the provision of drug samples to physicians, as well as prohibits states from licensing distributors of prescription drugs unless the state licensing program meets certain federal guidelines that include minimum standards for storage, handling and record keeping. In addition, the PDMA sets forth civil and criminal penalties for violations.

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Foreign regulation

In order to market any product outside of the United States, we would need to comply with numerous and varying regulatory requirements of other countries regarding safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of our products. Whether or not we obtain FDA approval for a product, we would need to obtain the necessary approvals by the comparable regulatory authorities of foreign countries before we can commence clinical trials or marketing of the product in those countries. The approval process varies from country to country and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country may negatively impact the regulatory process in others.

To date, we have not initiated any discussions with the European Medicines Agency or any other foreign regulatory authorities with respect to seeking regulatory approval for any of our products in Europe or in any other country outside the United States.

New legislation and regulations

From time to time, legislation is drafted, introduced and passed in Congress that could significantly change the statutory provisions governing the testing, approval, manufacturing and marketing of products regulated by the FDA. For example, the FDAAA discussed above was enacted in 2007. In addition to new legislation, FDA regulations and policies are often revised or interpreted by the agency in ways that may significantly affect our business and our products. It is impossible to predict whether further legislative changes will be enacted or whether FDA regulations, guidance, policies or interpretations changed or what the effect of such changes, if any, may be.

Pharmaceutical coverage, pricing and reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any drug products for which we obtain regulatory approval. Sales of any of our product candidates, if approved, will depend, in part, on the extent to which the costs of the products will be covered by third-party payors, including government health programs such as Medicare and Medicaid, commercial health insurers and managed care organizations. The process for determining whether a payor will provide coverage for a drug product may be separate from the process for setting the price or reimbursement rate that the payor will pay for the drug product once coverage is approved. Third-party payors may limit coverage to specific drug products on an approved list, or formulary, which might not include all of the approved drugs for a particular indication.

In order to secure coverage and reimbursement for any product that might be approved for sale, we may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of the product, in addition to the costs required to obtain FDA or other comparable regulatory approvals. Our product candidates may not be considered medically necessary or cost-effective. A payor s decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Third-party reimbursement may not be sufficient to enable us to maintain price levels high enough to realize an appropriate return on our investment in product development.

The containment of healthcare costs has become a priority of federal, state and foreign governments, and the prices of drugs have been a focus in this effort. Third-party payors are increasingly challenging the prices charged for medical products and services and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. If these third-party payors do not

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consider our products to be cost-effective compared to other available therapies, they may not cover our products after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow us to sell our products at a profit. The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost containment programs to limit the growth of government-paid health care costs, including price controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs. Adoption of such controls and measures, and tightening of restrictive policies in jurisdictions with existing controls and measures, could limit payments for pharmaceuticals such as the drug candidates that we are developing and could adversely affect our net revenue and results.

Pricing and reimbursement schemes vary widely from country to country. Some countries provide that drug products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular product candidate to currently available therapies. For example, the European Union provides options for its member states to restrict the range of drug products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. European Union member states may approve a specific price for a drug product or may instead adopt a system of direct or indirect controls on the profitability of the company placing the drug product on the market. Other member states allow companies to fix their own prices for drug products, but monitor and control company profits. The downward pressure on health care costs in general, particularly prescription drugs, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert competitive pressure that may reduce pricing within a country. There can be no assurance that any country that has price controls or reimbursement limitations for drug products will allow favorable reimbursement and pricing arrangements for any of our products.

The marketability of any products for which we receive regulatory approval for commercial sale may suffer if the government and third-party payors fail to provide adequate coverage and reimbursement. In addition, an increasing emphasis on managed care in the United States has increased and we expect will continue to increase the pressure on drug pricing. Coverage policies, third-party reimbursement rates and drug pricing regulation may change at any time. In particular, the Patient Protection and Affordable Care Act was enacted in the United States in March 2010 and contain provisions that may reduce the profitability of drug products, including, for example, increased rebates for drugs sold to Medicaid programs, extension of Medicaid rebates to Medicaid managed care plans, mandatory discounts for certain Medicare Part D beneficiaries and annual fees based on pharmaceutical companies share of sales to federal health care programs. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

EMPLOYEES

As of March 15, 2012, we had 21 full-time employees, including a total of ten employees with M.D. or Ph.D. degrees. Of these full-time employees, 12 employees are engaged in research and development activities. None of our employees is represented by a labor union or covered by a collective bargaining agreement. We consider our relationship with our employees to be good.

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OUR CORPORATE INFORMATION

We were incorporated under the laws of the State of Delaware in August 2010. Our principal executive offices are located at 215 First Street, Suite 440, Cambridge, Massachusetts 02142 and our telephone number is (617) 252-9300.

ADDITIONAL INFORMATION

We maintain a website at www.verastem.com. We make available, free of charge on our website, our annual report on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and all amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended, or the Exchange Act, as soon as reasonably practicable after we electronically file those reports with, or furnish them to, the Securities and Exchange Commission, or SEC. We also make available, free of charge on our website, the reports filed with the SEC by our executive officers, directors and 10% stockholders pursuant to Section 16 under the Exchange Act as soon as reasonably practicable after copies of those filings are provided to us by those persons. The information contained on, or that can be accessed through, our website is not a part of or incorporated by reference in this annual report on Form 10-K.

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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. Our net loss was $13.7 million for the year ended December 31, 2011. As of December 31, 2011, we had a deficit accumulated during the development stage of $14.5 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 formulation 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

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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.

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 that the net proceeds from our initial public offering completed in February 2012, together with 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;

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- 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.

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

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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 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

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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 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;

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- 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.

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;

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- 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.

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

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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.

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

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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.

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;

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- 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.

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.

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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 size 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 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.

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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 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.

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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 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.

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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.

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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 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.

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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.

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

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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.

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

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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 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

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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.

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

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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 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

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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.

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:

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- 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;
- 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;

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- 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.

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

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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 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

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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.

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 March 15, 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

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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.

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;

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- 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.

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 March 15, 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

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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.

Item 1B. Unresolved Staff Comments

None.

Item 2. Properties

We occupy approximately 7,484 square feet of office and laboratory space in Cambridge, Massachusetts under a lease that expires in October 2014. We believe that our facility is sufficient to meet our current needs and that suitable additional space will be available as and when needed.

Item 3. Legal Proceedings

None.

Item 4. Mine Safety Disclosures

None.

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PART II

Item 5. Market for Registrant s Common Equity, Related Stockholder Matters and Issuers Purchases of Equity Securities

MARKET INFORMATION

Our common stock has been publicly traded on the NASDAQ Global Market under the symbol VSTM since January 27, 2012. Prior to that time, there was no public market for our common stock. As a result, we have not set forth quarterly information with respect to the high and low prices for our common stock for the two most recent fiscal years.

HOLDERS

As of March 15, 2012, there were 55 holders of record of our common stock. This number does not include beneficial owners whose shares are held by nominees in street name.

DIVIDENDS

We have never declared or paid cash dividends on our common stock, and we do not expect to pay any cash dividends on our common stock in the foreseeable future.

SECURITIES AUTHORIZED FOR ISSUANCE UNDER EQUITY COMPENSATION PLANS

The following table contains information about our equity compensation plans as of December 31, 2011.

Plan category	Number of securities to be issued upon exercise of outstanding stock options, warrants and rights	Weighted-average exercise price of outstanding options, warrants and rights	Number of securities remaining available for future issuance under equity compensation plans (excluding securities reflected in column (a))
	405,141	$ 0.75	30,101

Equity compensation plans approved by security holders (1)				
Equity compensation plans not approved by security holders				
Total	405,141	$	0.75	30,101

(1) Includes information regarding our 2010 equity incentive plan.

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RECENT SALES OF UNREGISTERED SECURITIES

Set forth below is information regarding securities sold by us during 2011 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

In April 2011 we sold an aggregate of 12,000,000 shares of our series A preferred stock at a price per share of $1.00 for an aggregate purchase price of $12 million.

In April 2011, we sold an aggregate of 256,000 shares of our common stock at a price per share of $0.28 for an aggregate purchase price of $71,680.

In July 2011, we sold an aggregate of 16,025,000 shares of our series B preferred stock at a price per share of $2.00 for an aggregate purchase price of $32.1 million.

In November 2011, we sold an aggregate of 9,067,825 shares of our series C preferred stock at a price per share of $2.25 for an aggregate purchase price of $20.4 million.

In November 2011, we agreed to issue a warrant for the purchase of 142,857 shares of our common stock with an exercise price equal to the average closing price of our common stock during the five days preceding the date of issuance to Poniard Pharmaceuticals, Inc. upon achievement of a milestone.

No underwriters were involved in the foregoing sales of securities. The securities were issued to investors in reliance upon the exemption from the registration requirements of the Securities Act, as set forth in Section 4(2) under the Securities Act, including in some cases, Regulation D promulgated thereunder, relative to transactions by an issuer not involving any public offering, to the extent an exemption from such registration was required. All purchasers of shares of preferred stock described above represented to us in connection with their purchase that they were accredited investors and were acquiring the shares for their own account for investment purposes only and not with a view to, or for sale in connection with, any distribution thereof. The purchasers received written disclosures that the securities had not been registered under the Securities Act and that any resale must be made pursuant to a registration statement or an available exemption from such registration. Each share of our preferred stock converted into approximately 0.29 shares of our common stock upon the closing of our initial public offering on February 1, 2012.

Stock option and other equity awards

During 2011, we issued to certain employees, directors and consultants options to purchase an aggregate of 227,998 shares of common stock at a weighted-average exercise price of $1.12 per share. We also agreed to grant restricted stock units for an aggregate of 600,000 shares of our common stock, effective upon the closing of our initial public offering.

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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 Annual Report on Form 10-K.

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 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 $56.7 million, after deducting approximately $4.4 million in underwriting discounts and commissions and approximately $2.1 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.

As of March 15, 2012, we have used approximately $4.3 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 6. Selected Financial Data

You should read the following selected financial data together with our financial statements and the related notes appearing elsewhere in this Annual Report on Form 10-K and the Management s discussion and analysis of financial condition and results of operations section of this Annual Report on Form 10-K. We have derived the statements of operations data for the year ended December 31, 2011, for the period from August 4, 2010 (inception) to December 31, 2010 and for the period from August 4, 2010 (inception) to December 31,

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2011 and the balance sheet data as of December 31, 2011 and December 31, 2010 from our audited financial statements included in this Annual Report on Form 10-K. Our historical results for any prior period are not necessarily indicative of results to be expected in any future period.

Statement of operations data:	Year ended December 31, 2011	Period from August 4, 2010 (inception) to December 31, 2010	Period from August 4, 2010 (inception) to December 31, 2011
Operating expenses:			
Research and development	$ 9,883	$ 400	$ 10,283
General and administrative	3,815	384	4,199
Total operating expenses	13,698	784	14,482
Loss from operations	(13,698)	(784)	(14,482)
Interest income	15		15
Net loss	$ (13,683)	$ (784)	$ (14,467)
Accretion of preferred stock	(32)	(2)	(34)
Net loss applicable to common stockholders	$ (13,715)	$ (786)	$ (14,501)
Net loss per share applicable to common stockholders basic and diluted	$ (10.59)	$ (0.91)	$ (12.39)
Weighted-average number of common shares used in net loss per share applicable to common stockholders basic and diluted	1,295	850	1,171

Balance sheet data:	December 31, 2011	2010
	(in thousands)	
Cash, cash equivalents and investments	$ 56,805	$ 3,584
Working capital	44,795	3,228
Total assets	59,037	3,604
Redeemable convertible preferred stock	68,141	3,923
Deficit accumulated during the development stage	(14,467)	(784)
Total stockholders deficit	(12,766)	(687)

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Item 7. 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 Annual Report on Form 10-K. Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report on Form 10-K, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. You should read the Risk factors section of this Annual Report on Form 10-K for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

OVERVIEW

We are a biopharmaceutical company focused on 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 expect to initiate clinical trials with VS-507 and one of either VS-4718 or VS-5095 over the next 12 to 15 months.

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.

As of December 31, 2011, we had a deficit accumulated during the development stage of $14.5 million. Our net loss was $13.7 million for the year ended December 31, 2011, $784,000 for the period from August 4, 2010 (inception) to December 31, 2010 and $14.5 million for the period from August 4, 2010 (inception) to December 31, 2011. 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.

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FINANCIAL OPERATIONS OVERVIEW

Revenue

To date, we have not generated any revenues. 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.

Research and development expenses

Research and development expenses consist of costs associated with our research activities, including our drug discovery efforts, and the development of our therapeutic product candidates and companion diagnostics. Our research and development expenses consist of:

- employee-related expenses, including salaries, benefits, travel and stock-based compensation expense;

- external research and development expenses incurred under arrangements with third parties, such as contract research organizations, or CROs, manufacturing organizations and consultants, including our scientific advisory board;

- license fees; and

- facilities, depreciation and other allocated expenses, which include direct and allocated expenses for rent and maintenance of facilities, depreciation of leasehold improvements and equipment, and laboratory and other supplies.

We expense research and development costs to operations as incurred. We account for nonrefundable advance payments for goods and services that will be used in future research and development activities as expenses when the service has been performed or when the goods have been received, rather than when the payment is made.

We use our employee and infrastructure resources across multiple research and development projects. We do not allocate employee-related expenses or depreciation to any particular project. Because all of our development projects are in preclinical development, we do not track research and development costs by project. The components of our research and development costs are described in more detail in Results of operations. We expect to begin to track specific project costs when product candidates enter toxicology studies to enable the filing of an IND with the FDA.

We anticipate that our research and development expenses will increase significantly in future periods as we increase the scope and rate of our drug discovery efforts and begin costlier development activities, including clinical trials for our current and additional product candidates in the future.

Our most advanced product candidates are VS-507, VS-4718 and VS-5095. We are currently evaluating these compounds in preclinical studies as potential therapies for breast and other cancers. We initiated IND-enabling toxicology studies for VS-507 in January 2012. Assuming successful completion of preclinical studies, we expect to initiate clinical trials with VS-507 and one of either VS-4718 or VS-5095 over the next 12 to 15 months.

The successful development of our product candidates is highly uncertain. As this time, we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete development of our product candidates or the period, if any, in which material net cash inflows from our product candidates may commence. This is due to the numerous risks and uncertainties associated with developing drugs, including the uncertainty of:

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- the scope, rate of progress and expense of our drug discovery efforts and other research and development activities;

- the potential benefits of our product candidates over other therapies;

- our ability to market, commercialize and achieve market acceptance for any of our product candidates that we are developing or may develop in the future;

- clinical trial results;

- the terms and timing of regulatory approvals; and

- the expense of filing, prosecuting, defending and enforcing patent claims and other intellectual property rights.

A change in the outcome of any of these variables with respect to the development of a product candidate could mean a significant change in the costs and timing associated with the development of that product candidate. For example, if the FDA or other regulatory authority were to require us to conduct clinical trials beyond those which we currently anticipate will be required for the completion of clinical development of a product candidate or if we experience significant delays in enrollment in any clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development.

General and administrative expenses

General and administrative expenses consist primarily of salaries and related costs for personnel, including stock-based compensation expense, in our executive, finance and business development functions. Other general and administrative expenses include allocated facility costs and professional fees for legal, patent, investor and public relations, consulting and accounting services.

We anticipate that our general and administrative expenses will increase in future periods to support increases in our research and development activities and as a result of increased headcount, expanded infrastructure, increased legal, compliance, accounting and investor and public relations expenses associated with being a public company and increased insurance premiums, among other factors.

Interest income

Prior to September 30, 2011, our cash and cash equivalents were invested in non-interest-bearing accounts. As a result, we only earned interest during the last three months of 2011. We expect interest income to increase in future periods as we invest the proceeds from our preferred stock financings and initial public offering.

Accretion of preferred stock

Prior to the conversion of our preferred stock into 11,740,794 shares of common stock upon the closing of our initial public offering in February 2012, our preferred stock was redeemable beginning in 2016 at its original issue price plus any declared but unpaid dividends upon a specified vote of the preferred stockholders. Accretion of preferred stock reflects the periodic accretion of issuance costs on our preferred stock to its redemption value.

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CRITICAL ACCOUNTING POLICIES AND SIGNIFICANT JUDGMENTS AND ESTIMATES

Our management s discussion and analysis of our financial condition and results of operations is based on our financial statements, which we have prepared in accordance with U.S. generally accepted accounting principles. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities and expenses and the disclosure of contingent assets and liabilities in our financial statements. On an ongoing basis, we evaluate our estimates and judgments, including those related to accrued expenses and stock-based compensation described in greater detail below. We base our estimates on our limited historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

Our significant accounting policies are described in more detail in the notes to our financial statements appearing elsewhere in this Annual Report on Form 10-K. However, we believe that the following accounting policies are the most critical to aid you in fully understanding and evaluating our financial condition and results of operations.

Accrued research and development expenses

As part of the process of preparing our financial statements, we are required to estimate our accrued expenses. This process involves reviewing quotations and contracts, identifying services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of the actual cost. The majority of our service providers invoice us monthly in arrears for services performed or when contractual milestones are met. We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of our estimates with the service providers and make adjustments if necessary. The significant estimates in our accrued research and development expenses include fees paid to CROs in connection with research and development activities for which we have not yet been invoiced.

We base our expenses related to CROs on our estimates of the services received and efforts expended pursuant to quotes and contracts with CROs that conduct research and development on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the research and development expense. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or prepaid accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and could result in us reporting amounts that are too high or too low in any particular period.

Stock-based compensation

Prior to becoming a public company, we utilized significant estimates and assumptions in determining the fair value of our common stock. We granted stock options at exercise prices not less than the fair market value of our common stock as determined by the board of directors, with

input from management. The board of directors determined the estimated fair value of our common stock based on a number of objective and subjective factors, including external market conditions affecting the biotechnology industry sector and the prices at which we sold

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shares of redeemable convertible preferred stock, the superior rights and preferences of securities senior to our common stock at the time and the likelihood of achieving a liquidity event, such as an initial public offering or sale of our company.

We utilized various valuation methodologies in accordance with the framework of the 2004 American Institute of Certified Public Accountants Technical Practice Aid, *Valuation of Privately-Held Company Equity Securities Issued as Compensation*, to estimate the fair value of our common stock. The methodologies included an asset-based approach and the current value method for our initial common stock valuation as of November 30, 2010, the option pricing method utilizing the reverse backsolve method to estimate our underlying equity value as of July 31, 2011 and a methodology that determined an estimated value under an initial public offering scenario and a sale scenario based upon an assessment of the probability of occurrence of each scenario as of September 30, 2011, November 17, 2011, and December 31, 2011. Each valuation methodology included estimates and assumptions that required our judgment. These estimates included assumptions regarding future performance, including the successful completion of preclinical studies and clinical trials and the time to completing an initial public offering or sale. Significant changes to the key assumptions used in the valuations could have resulted in different fair values of common stock at each valuation date.

RESULTS OF OPERATIONS

We were incorporated on August 4, 2010. As a result, our results of operations reflect the year ended December 31, 2011 and the period from August 4, 2010 (inception) to December 31, 2010. There is no comparable period for 2010.

Discussion of the year ended December 31, 2011

Research and development expenses. Research and development expenses were $9.9 million for the year ended December 31, 2011. Expenses during the year included:

- Contract research organization expenses of $3.7 million, representing 37% of total research and development expenses during the year, comprised of expenses for outsourced biology, chemistry and development services.

- Payroll expense of $1.5 million, representing 15% of total research and development expenses during the year, including salaries, payroll taxes and benefits for our employees in research and development. We had 11 employees in research and development at December 31, 2011. Payroll expense also included stock-based compensation expense for employees of $30,000.

- Consulting fees of $1.3 million, representing 13% of total research and development expenses during the year, including $476,000 for our scientific advisory board, $232,000 for recruitment consultants and $143,000 for database consultants.

- Laboratory supply expense of $1.0 million, representing 11% of total research and development expenses during the year.

- Non-employee stock-based compensation expense of $1.0 million, representing 10% of total research and development expenses during the year, related to stock options and restricted stock awarded to members of our scientific advisory board.

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- License fee expense of $842,000, representing 9% of total research and development expense during the year, comprised of upfront and annual license fees, including $406,000 for the obligation to issue a warrant for the purchase of 142,857 shares of our common stock to Poniard Pharmaceuticals, Inc.

- Occupancy expense of $465,000, representing 5% of total research and development expenses during the year, which is an allocated portion of rent and other occupancy costs.

General and administrative expenses. General and administrative expenses were $3.8 million for the year ended December 31, 2011. Expenses during the year included:

- Payroll expense of $1.4 million, representing 38% of total general and administrative expenses during the year, including salaries, payroll taxes and benefits for our general and administrative employees. Payroll expense included stock-based compensation expense for employees of $62,000.

- Consulting fees of $696,000, representing 18% of total general and administrative expenses during the year, including business development, public relations and finance consultants.

- Professional fee expense of $666,000, representing 18% of total general and administrative expenses during the year, comprised of fees for audit, tax and legal services, including the reimbursement to the Whitehead Institute of patent costs related to our licenses with the Whitehead Institute.

- Non-employee stock-based compensation expense of $592,000, representing 16% of total general and administrative expenses during the year, related to restricted stock awarded to our co-founders.

- Occupancy expense of $207,000, representing 5% of total general and administrative expenses during the year, which is an allocated portion of rent and other occupancy costs.

- Travel expense of $190,000, representing 5% of total general and administrative expenses during the year, including travel, meals, entertainment and conferences.

Interest income. We recorded $15,000 of interest income in the year ended December 31, 2011 associated with our cash equivalents and investments.

Accretion of preferred stock. We recorded $32,000 of accretion in the year ended December 31, 2011 reflecting the periodic accretion of issuance costs associated with our series A, series B and series C preferred stock.

Discussion of the period from August 4, 2010 (inception) to December 31, 2010

Research and development expenses. Research and development expenses were $400,000 for the period from August 4, 2010 (inception) to December 31, 2010. Expenses during the period included:

- License fee expense of $182,000, representing 46% of total research and development expenses during the period, comprised of fees for our exclusive and non-exclusive licenses, as well as the fair value of common stock that we issued to the Whitehead Institute in connection with our drug discovery platform license agreement.

- Consulting fees of $137,000, representing 34% of total research and development expenses during the period, primarily for our scientific advisory board.

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- Contract research organization expenses of $42,000, representing 11% of total research and development expenses during the period, including expenses for outsourced biology and chemistry.

- Non-employee stock-based compensation expense of $24,000, representing 6% of total research and development expenses during the period, related to stock options and restricted stock awarded to members of our scientific advisory board.

- Laboratory supply expense of $13,000, representing 3% of total research and development expenses during the period.

General and administrative expenses. General and administrative expenses were $384,000 for the period from August 4, 2010 (inception) to December 31, 2010. Expenses during the period included:

- Professional fee expense of $182,000, representing 48% of total general and administrative expenses during the period, comprised of fees for audit, tax and legal services, including the reimbursement to the Whitehead Institute of patent costs related to our drug discovery platform license agreement.

- Payroll expense of $96,000, representing 25% of total general and administrative expenses during the period, including salaries, payroll taxes and benefits for our general and administrative employees. Stock-based compensation expense was not material to the financial statements.

- Occupancy expense of $36,000, representing 9% of total general and administrative expenses during the period, which is an allocated portion of rent and other occupancy costs.

- Non-employee stock-based compensation expense of $28,000, representing 7% of total general and administrative expenses during the period, related to restricted stock awarded to our co-founders.

- Consulting fees of $26,000, representing 7% of total general and administrative expenses during the period, including business development, public relations and information technology consultants.

- Travel expense of $16,000, representing 4% of total general and administrative expenses during the period, including travel, meals, entertainment and conferences.

Accretion of preferred stock. We recorded $2,000 of accretion in the period from August 4, 2010 (inception) to December 31, 2010 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. We have financed our operations to date through private placements of our preferred stock and our initial public offering, which we completed in February 2012. As of December 31, 2011, we had received $68.1 million in net proceeds from the issuance of preferred stock. As of December 31, 2011, we had $21.0 million in cash and cash equivalents, $26.9 million in short-term investments and $9.0 million in long-term investments. In February 2012, we received $56.7 million in net proceeds from our initial public offering. We primarily invest our cash, cash equivalents and investments in a U.S. Treasury money market fund, U.S. agency notes and corporate bonds.

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Cash flows

The following table sets forth the primary sources and uses of cash for each of the periods set forth below.

(in thousands)	Year ended December 31, 2011	Period from August 4, 2010 (inception) to December 31, 2010
Net cash used in operating activities	$ (10,132)	$ (330)
Net cash used in investing activities	(36,722)	(8)
Net cash provided by financing activities	64,224	3,922
Net increase in cash and cash equivalents	$ 17,370	$ 3,584

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 year ended December 31, 2011 compared to the period from August 4, 2010 (inception) to December 31, 2010 is due to an increase in research and development expenses as we increased our research and development headcount, increased spending on external research and development costs and increases in the balance of accounts payable, accrued expenses and deferred rent. In addition, we commenced operations in August 2010 and, as such, the period ended December 31, 2010 reflects only five months of activity. We expect cash used in operating activities to continue to increase for the foreseeable future as we fund our increased research and development activities.

Investing activities. The cash used in investing activities for all periods reflects the purchases of property and equipment. The majority of such purchases in the year ended December 31, 2011 were for laboratory equipment. In addition, during the year ended December 31, 2011, investing activities included $35.9 million used to purchase investments and an $86,000 increase in restricted cash related to a standby letter of credit issued as a security deposit for our facility lease.

Financing activities. The cash provided by financing activities in the year ended December 31, 2011 is the result of the sale and issuance of 12,000,000 shares of our series A preferred stock for net proceeds of $12.0 million, the sale and issuance of 16,025,000 shares of our series B preferred stock for net proceeds of $31.9 million, the sale and issuance of 9,067,825 shares of our series C preferred stock for net proceeds of $20.2 million and $38,000 of net proceeds from the sale of restricted stock to employees. The cash provided by financing activities in the period from August 4, 2010 (inception) to December 31, 2010 is primarily the result of the sale and issuance of 4,000,000 shares of our series A preferred stock for net proceeds of $3.9 million.

Funding requirements

All of our product candidates are still in preclinical development. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. 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;

- acquire or in-license other products and technologies;

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- 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.

We expect that the net proceeds from our initial public offering in February 2012, together with our existing cash, cash equivalents and investments, will enable us to fund our current operating plan and capital expenditure requirements into 2016. We have based this estimate on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates, and the extent to which we may enter into collaborations with third parties for development and commercialization of our product candidates, we are unable to estimate the amounts of increased capital outlays and operating expenses associated with completing the development of our current product candidates. 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.

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

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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 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.

CONTRACTUAL OBLIGATIONS AND COMMITMENTS

The following table summarizes our contractual obligations at December 31, 2011.

(in thousands)	Total	Less than 1 year	1-3 years	3-5 years	More than 5 years
Operating lease obligations	$ 1,018	$ 351	$ 667		
License agreements(1)					

(1) As discussed in Note 10 to the financial statements appearing elsewhere in this Annual Report on Form 10-K, we have executed several agreements to license intellectual property. The license agreements require us to pay upfront license fees and ongoing annual license maintenance fees, totaling a minimum of $95,000 per year beginning in 2012 up to a maximum amount of $155,000 per year beginning in 2015, as well as reimburse certain patent costs previously incurred by the licensors, as applicable. We have not included maintenance fees in the table above since the minimum annual payments are perpetual and the agreements are cancelable by us at any time upon 90 days prior written notice to the licensor.

Under our drug discovery platform license agreement, which we amended and restated in January 2012, we also have agreed to make milestone payments to the Whitehead Institute upon achieving various development, regulatory and commercialization milestones. For each licensed product, we agreed to make milestone payments of up to an aggregate of $1,560,000 plus an additional amount for each subsequent approval of additional indications for a maximum number of licensed products. For each identified product that is not a licensed product, we agreed to make milestone payments of up to an aggregate of $815,000 plus an additional amount for each subsequent approval of additional indications for a maximum number of identified products. Each type of specified milestone payment is payable only for each of the maximum number of licensed products and the maximum number of identified products, as the case may be, to achieve the applicable milestone. In addition, a separate milestone payment is due upon the first commercial sale of each licensed product or identified product that is a diagnostic or prognostic test. A single additional milestone payment is due for the first issuance of licensed patent rights in the United States, the United Kingdom, France, Germany, Spain or Italy. In addition, we have agreed to pay the Whitehead Institute royalties as a percentage of net sales of licensed products. The royalty rate is in the low single digits as a percentage of net sales for licensed products that are therapeutics, the mid single digits for licensed products that are diagnostics or prognostics and less than one percent for identified products.

Under our license agreement with Poniard Pharmaceuticals, Inc., or Poniard, that we entered into in November 2011 relating to VS-4718 and VS-5095 and other compounds covered by a licensed patent right under that agreement that have the inhibition of Focal Adhesion Kinase as a primary mode of action, we paid an upfront license fee and agreed to pay Poniard milestone payments of up to an aggregate of $13,250,000 upon the achievement of specified development and regulatory milestones. We also agreed to issue to Poniard a warrant

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to purchase 142,857 shares of our common stock upon the first dosing of the first patient in our first Phase 1 clinical trial of a licensed product. The exercise price of such warrant would be equal to the average closing price of our common stock during the five trading days preceding such issue date. In addition, we agreed to pay low to mid single digit royalties to Poniard as a percentage of net sales of licensed products.

Under our separate exclusive license agreement with the Whitehead Institute, or the cancer diagnostic license agreement, which we amended and restated in December 2011, we paid an upfront license fee and agreed to make milestone payments of up to an aggregate of $825,000 to the Whitehead Institute upon achieving specified regulatory and commercialization milestones. In addition, we have agreed to pay the Whitehead Institute royalties as a percentage of net sales of licensed products. The royalty rate is in the mid single digits as a percentage of net sales.

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.

TAX LOSS CARRYFORWARDS

As of December 31, 2011, we had federal net operating loss carryforwards of $11.6 million and state net operating loss carryforwards of $11.9 million , which are available to reduce future taxable income. We also had federal tax credits of $358,000 and state tax credits of $42,000, which may be used to offset future tax liabilities. The net operating loss and tax credit carryforwards will expire at various dates through 2031. Net operating loss and tax credit carryforwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities and may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant stockholders over a three-year period in excess of 50%, as defined under Sections 382 and 383 of the Internal Revenue Code, as well as similar state provisions. This could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on the value of our company immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation in future years. At December 31, 2011, we recorded a 100% valuation allowance against our net operating loss and tax credit carryforwards of approximately $5.2 million, as we believe it is more likely than not that the tax benefits will not be fully realized. In the future, if we determine that a portion or all of the tax benefits associated with our tax carryforwards will be realized, net income would increase in the period of determination.

RECENTLY ADOPTED ACCOUNTING STANDARDS

We have not recently adopted any new accounting standards.

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. The provisions of this ASU are effective prospectively for interim and annual periods beginning on or after December 15, 2011. Early application is prohibited. We do not expect the adoption of these provisions to have a significant impact on our financial statements.

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In June 2011, the FASB issued ASU No. 2011-05, Comprehensive Income. This ASU intends to enhance 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 deficit. The provisions of this ASU will be applied retrospectively for interim and annual periods beginning after December 15, 2011. Early application is permitted. We do not expect the adoption of these provisions to have a significant impact on our financial statements , but it will impact the manner in which we present comprehensive income (loss).

Item 7A. Quantitative and Qualitative Disclosures About Market Risk

We are exposed to market risk related to changes in interest rates. We had cash and cash equivalents of $3.6 million as of December 31, 2010. We had cash, cash equivalents and investments of $56.8 million as of December 31, 2011, consisting of cash, money market funds, corporate debt securities and United States Treasuries and agencies. 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 December 31, 2011, approximately $7,000 of our total liabilities was denominated in currencies other than the functional currency. As of December 31, 2010, all of our liabilities were denominated in our functional currency.

Item 8. Financial Statements and Supplementary Data

Our financial statements, together with the report of our independent registered public accounting firm, appear on pages F-1 through F-27 of this Annual Report on Form 10-K.

Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure

None.

Item 9A. 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 December 31, 2011. 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

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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 December 31, 2011, 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.

Management s Annual Report on Internal Control Over Financial Reporting

This Annual Report on Form 10-K does not include a report of management s assessment regarding internal control over financial reporting or an attestation report of the company s independent registered public accounting firm due to a transition period established by rules of the Securities and Exchange Commission for newly public companies.

Changes in Internal Control Over Financial Reporting

No change in our internal control over financial reporting occurred during the fiscal quarter ended December 31, 2011 that has materially affected, or is reasonably likely to materially affect, the Company s internal control over financial reporting.

Item 9B. Other Information

On March 28, 2012, we entered into an employment agreement with Paul Brannelly, one of our named executive officers for the year ended December 31, 2011 and our current Vice President of Finance. The terms of the employment agreement are described in Executive Compensation in Item 5 of this Annual Report on Form 10-K.

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PART III

Item 10. Directors, Executive Officers and Corporate Governance

Directors and Executive Officers

The following table sets forth the name, age and position of each of our directors and executive officers as of March 15, 2012.

Name	Age	Position
Christoph Westphal, M.D., Ph.D.(2)	43	President, Chief Executive Officer and Director
Robert Forrester	48	Chief Operating Officer
Jonathan Pachter, Ph.D.	54	Vice President, Head of Research
Richard Aldrich(2)(3)	57	Director
John K. Clarke(1)	58	Director
Ansbert Gadicke, M.D.(2)	54	Director
Stephen Kraus(1)(3)	35	Director
Henri Termeer(1)(3)	66	Director

(1) Member of the audit committee.

(2) Member of the nominating and corporate governance committee.

(3) Member of the compensation committee.

Christoph Westphal, M.D., Ph.D. has served as our President and Chief Executive Officer since September 2011. He has served on our board of directors since August 2010 and as the Chairman of our board of directors since March 2011. Dr. Westphal has served as a partner of Longwood Fund, LP, a venture capital investment fund, since 2010. He served as the President of SR One, the corporate venture capital arm of GlaxoSmithKline, from 2010 until 2011. Dr. Westphal has previously been involved in founding a number of biotechnology companies as chief executive officer. Dr. Westphal co-founded Sirtris Pharmaceuticals, Inc., which was acquired by GlaxoSmithKline plc in 2008, and served as its Chief Executive Officer from 2004 to 2010. He also co-founded Alnara Pharmaceuticals, Inc., Acceleron Pharma, Inc., serving as its Chief Executive Officer in 2003, Alnylam Pharmaceuticals, Inc., serving as its Chief Executive Officer in 2002, and Momenta Pharmaceuticals, Inc., serving as its Chief Executive Officer in 2001. Dr. Westphal serves on the Board of Fellows of Harvard Medical School and the Board of Overseers for the Boston Symphony Orchestra and is a member of the Research Advisory Council at the Massachusetts General Hospital. He earned his M.D. from Harvard Medical School, his Ph.D. in genetics from Harvard University and his B.A. from Columbia University. We believe that Dr. Westphal is qualified to serve on our board of directors due to his experience in the life sciences industry as an entrepreneur and venture capitalist and his service on the boards of directors of other life sciences companies.

Robert Forrester has served as our Chief Operating Officer since March 2011. Mr. Forrester has previously held executive level positions at both private and public life sciences companies. Prior to joining us, Mr. Forrester served as Chief Operating Officer of Forma Therapeutics, Inc. from 2010 until 2011. Previously he served as Interim President and Chief Executive Officer of CombinatoRx, Inc., now Zalicus Inc., from 2009 until 2010 and as its Executive Vice President and Chief Financial Officer from 2004 to 2009. Mr. Forrester

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served as Senior Vice President, Finance and Corporate Development at Coley Pharmaceuticals Group, Inc. from 2000 to 2003. He earned his LL.B. from Bristol University in England.

Jonathan Pachter, Ph.D. has served as our Vice President, Head of Research since July 2011. Prior to joining us, Dr. Pachter served as the Senior Director of Cancer Biology at OSI Pharmaceuticals, Inc., which was acquired by Astellas Pharma Inc. in 2010, from 2005 to 2011. He earned his Ph.D. in Neuroscience and his M.S. in Pharmacology from Baylor College of Medicine.

Richard Aldrich has served as a member of our board of directors since August 2010. Mr. Aldrich has served as a partner of Longwood Fund, LP, a venture capital investment fund, since 2010. He founded RA Capital Management LLC, a hedge fund, in 2004 and served as a Managing Member from 2004 to 2008 and as a Co-Founding Member from 2008 until 2011. He co-founded Sirtris Pharmaceuticals, Inc., which was acquired by GlaxoSmithKline plc in 2008, and served on its board of directors from 2004 to 2008; co-founded Concert Pharmaceuticals, Inc. and has served as chairman of its board of directors since 2006; and co-founded Alnara Pharmaceuticals, Inc. and served on its board of directors from 2008 to 2010. Mr. Aldrich also joined Vertex Pharmaceuticals, Inc. at its founding in 1989 and served as its Senior Vice President and Chief Business Officer until 2001. He earned his M.B.A from the Amos Tuck School at Dartmouth College and his B.S. from Boston College. We believe that Mr. Aldrich is qualified to serve on our board of directors due to his experience in the life sciences industry as an entrepreneur and venture capitalist and his service on the boards of directors of other life sciences companies.

John K. Clarke has served as a member of our board of directors since November 2010. Mr. Clarke co-founded Cardinal Partners, a venture capital firm, and has served as its Managing General Partner since 1997. Mr. Clarke co-founded Alnylam Pharmaceuticals, Inc. and has served on its board of directors since 2002. He also serves on the board of directors of Momenta Pharmaceuticals, Inc. Mr. Clarke also co-founded and has served as chief executive officer for a number of other companies, including Alkermes, Inc., Arris Pharmaceuticals, Inc., Cubist Pharmaceuticals, Inc. and the DNX Corporation. He earned his M.B.A. from the Wharton School of the University of Pennsylvania and his B.A. in Biology and Economics from Harvard College. We believe that Mr. Clarke is qualified to serve on our board of directors due to his financial expertise, years of experience providing advisory services to organizations in the life sciences industry and his service on the boards of directors of other life sciences companies.

Ansbert Gadicke, M.D. has served as a member of our board of directors since November 2010. Dr. Gadicke co-founded MPM Group, a venture capital firm, and has served as the managing director of MPM Asset Management LLC since 1996. He serves on the board of directors of Radius Health, Inc. and a number of privately-held life sciences companies. Dr. Gadicke previously served as a member of the board of directors of Pharmasset, Inc. from 1999 until 2007 and as a member of the board of directors of PharmAthene, Inc. from 2004 until 2007. Dr. Gadicke also serves on the Board of Fellows of Harvard Medical School. He earned his M.D. from J.W. Goethe University in Frankfurt. We believe that Dr. Gadicke is qualified to serve on our board of directors due to his experience in the life sciences industry as a venture capitalist, his training as a physician and his service on the boards of directors of other life sciences companies.

Stephen Kraus has served as a member of our board of directors since November 2010. Mr. Kraus has served as an investment professional at Bessemer Venture Partners, a venture capital firm, since 2004 and has been employed as a Partner since 2010. He serves on the board of directors of a number of privately-held life sciences companies. He previously served as a member of the board of directors of Sirtris Pharmaceuticals, Inc. from 2005 until 2007 and as a member of the board of directors of Restore Medical, Inc. from 2005 until 2008. He earned his M.B.A. from Harvard Business School and his B.A. from Yale University. We believe that

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Mr. Kraus is qualified to serve on our board of directors due to his experience in the life sciences industry as a venture capitalist and his service on the boards of directors of other life sciences companies.

Henri Termeer has served as a member of our board of directors since June 2011. Mr. Termeer served as President and a member of the board of directors of Genzyme Corporation from 1983 until its acquisition by sanofi-aventis U.S., LLC in 2011, its Chief Executive Officer from 1985 to 2011 and the chairman of its board of directors from 1988 to 2011. He serves on the Council of Economic Advisors to Massachusetts Governor Deval Patrick and as co-chair of the Leadership Counsel of the Massachusetts Life Sciences Collaborative. Mr. Termeer is also chairman emeritus of the New England Healthcare Institute and a trustee for the Boston Museum of Science. Mr. Termeer serves on the board of directors of ABIOMED Inc., AVEO Pharmaceuticals, Inc., Massachusetts General Hospital, the Massachusetts Institute of Technology Corporation and Partners HealthCare, and, until December 31, 2011, served as chairman of the board of directors of the Federal Reserve Bank of Boston. Mr. Termeer also serves on the Board of Fellows of Harvard Medical School. He earned his M.B.A. from the Darden School at the University of Virginia. We believe Mr. Termeer is qualified to serve on our board of directors due to his senior executive experience in developing and managing Genzyme Corporation over the course of many years, his service on the boards of directors of Genzyme Corporation and other life sciences companies and his deep life sciences industry experience and knowledge.

Family Relationships

There are no family relationships among any of our directors or executive officers.

Section 16(a) Beneficial Ownership Reporting Compliance

Our directors, executive officers and beneficial owners of more than 10% of our common stock are required under Section 16(a) of the Securities Exchange Act of 1934, as amended, to file reports of ownership and changes in ownership of our securities with the SEC. We completed the initial public offering of our common stock on February 1, 2012, and accordingly, we did not have a class of securities registered pursuant to Section 12 of the Exchange Act in 2011.

Code of Business Conduct and Ethics

We have adopted a written code of business conduct and ethics that applies to our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. A current copy of the code is posted on the Investors Corporate Governance section of our website, which is located at www.verastem.com. In addition, we intend to post on our website all disclosures that are required by law, the rules of the SEC or NASDAQ stock market listing standards concerning any amendments to, or waivers from, any provision of the code.

Board Committees

Our board of directors has established an audit committee, a nominating and corporate governance committee and a compensation committee, each of which operates under a charter that has been approved by our board. Our board of directors has determined that all of the members of the audit committee, the compensation committee and the nominating and corporate governance committee, other than Dr. Westphal, are independent as defined under NASDAQ Marketplace Rules, including, in the case of all the members of our audit committee, the independence requirements contemplated by Rule 10A-3 under the Securities Exchange Act of 1934.

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Audit committee

The members of our audit committee are Mr. Clarke, Mr. Kraus and Mr. Termeer. Mr. Clarke chairs the audit committee. Our board of directors has determined that Mr. Clarke is an audit committee financial expert as defined in applicable SEC rules.

Nominating and corporate governance committee

The members of our nominating and corporate governance committee are Mr. Aldrich, Dr. Gadicke, and Dr. Westphal. Mr. Aldrich chairs the nominating and corporate governance committee.

No changes have been made to the procedures by which our stockholders may recommend nominees to our board of directors.

Compensation committee

The members of our compensation committee are Mr. Termeer, Mr. Aldrich and Mr. Kraus. Mr. Termeer chairs the compensation committee.

Item 11. Executive Compensation

COMPENSATION DISCUSSION AND ANALYSIS

Overview

This section discusses the principles underlying our policies and decisions with respect to the compensation of our executive officers and what we believe are the most important factors relevant to an analysis of these policies and decisions. This section also describes the material elements of compensation awarded to, earned by or paid to each of our named executive officers for 2011. Our named executive officers for 2011 consist of our three current executive officers, Christoph Westphal, M.D., Ph.D., our President and Chief Executive Officer, Robert Forrester, our Chief Operating Officer who also serves as our principal financial officer, and Jonathan Pachter, Ph.D., our Vice President, Head of Research; and three individuals who previously served as executives officers with us, Paul Brannelly, our current Vice President of Finance who served as our principal financial officer prior to the arrival of Mr. Forrester, Satish Jindal, Ph.D., our former President and Chief Operating Officer who remains with us as a non-executive employee, and Peter Elliott, Ph.D., our former Head of Research and Development. In addition, this section provides qualitative information regarding the manner and context in which compensation is awarded to and earned by our executive officers and is intended to provide context for the data presented in the tables and narrative that follow.

We commenced operations in November 2010 and hired each of our current executive officers in 2011. Dr. Westphal, our President and Chief Executive Officer, does not currently receive, and has not historically received, any compensation from us for his service as President and Chief Executive Officer because of his service as a general partner of Longwood Fund, LP, a venture capital investment fund and one of our principal stockholders. Our compensation committee is currently evaluating potential compensation arrangements for Dr. Westphal designed to align Dr. Westphal s interests with those of our stockholders and provide Dr. Westphal with an incentive to remain in his position as our President and Chief Executive Officer. The compensation of each of our other current executive officers is based on individual terms approved by our board of directors at the time of hire. Following our initial public offering, or IPO, in February 2012, our compensation committee

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began overseeing our compensation policies and, together with our board of directors, will periodically evaluate the need for revisions to ensure our compensation program is competitive with the companies with which we compete for executive talent.

Objectives and philosophy of our executive compensation program

The primary objectives of our executive compensation program are to:

- attract, retain and motivate experienced and talented executives;
- ensure executive compensation is aligned with our corporate strategies, research and development programs and business goals;
- recognize the individual contributions of executives while fostering a shared commitment among executives by aligning their individual goals with our corporate goals;
- promote the achievement of key strategic, development and operational performance measures by linking compensation to the achievement of measurable corporate and individual performance goals; and
- align the interests of our executives with our stockholders by rewarding performance that leads to the creation of stockholder value.

Each of our named executive officers was hired by us before our board of directors established a formal executive compensation program. To achieve these objectives, our board of directors and compensation committee are evaluating our executive compensation program with the goal of setting and maintaining compensation at levels that are justifiable based on each executive s level of experience, performance and responsibility and that our board of directors and compensation committee believe are competitive with those of other companies in our industry and our region that compete with us for executive talent. In addition, beginning in 2012 our executive compensation program will tie a substantial portion of each executive s overall compensation to key strategic, financial and operational goals. We have provided, and expect to continue to provide, a portion of our executive compensation in the form of stock options, restricted stock and restricted stock units that vest over time, which we believe helps to retain our executives and aligns their interests with those of our stockholders by allowing them to participate in the longer term success of our company as reflected in stock price appreciation.

Use of compensation consultants and market benchmarking

For purposes of determining total compensation and the primary components of compensation for our executive officers in 2011, we did not retain the services of a compensation consultant or use survey information or compensation data to engage in benchmarking. Beginning with 2012 compensation, our compensation committee is considering publicly available compensation data for national and regional companies in the biotechnology industry to help guide its executive compensation decisions at the time of hiring and for subsequent adjustments in compensation. In connection with designing our compensation program, our board of directors recently retained the services of Pearl Meyer & Partners, or Pearl Meyer, an independent compensation consultant, to provide additional comparative data on executive compensation practices in our industry and to advise on our executive compensation program generally. Although we expect that our board of directors and compensation committee will consider Pearl Meyer s advice and recommendations about our

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executive compensation program, the board of directors and compensation committee will ultimately make their own decisions about these matters.

We anticipate that Pearl Meyer will provide our board of directors and compensation committee with comparative data showing where our total compensation and each element of our compensation rate among both public and private companies in the biotechnology and life sciences industry generally and a peer group of publicly-traded companies in the life science industry at a stage of development, market capitalization and size comparable to ours with which the board of directors and compensation committee believe we compete for executive talent. We currently expect that the companies to be included in this peer group will be:

Aegerion Pharmaceuticals, Inc.

Alnylam Pharmaceuticals, Inc.

Amicus Therapeutics, Inc.

Anacor Pharmaceuticals, Inc.

Anthera Pharmaceuticals, Inc.

ARIAD Pharmaceuticals, Inc.

Aveo Pharmaceuticals, Inc.

Curis Inc.

Cytokinetics, Inc.

Endocyte, Inc.

Infinity Pharmaceuticals, Inc.

Ironwood Pharmaceuticals, Inc.

Myrexis, Inc.

Osiris Therapeutics, Inc.

Synta Pharmaceuticals Corp.

Zalicus Inc.

This peer group is subject to change, and we anticipate that our board of directors and compensation committee will periodically review and update the list. The peer group will be used for purposes of gathering data to help develop our executive compensation practices and guide our compensation decisions. We also expect that Pearl Meyer will make suggestions about our executive compensation practices based on the data it provides to us as well as compensation trends in our industry. We expect that the board of directors and compensation committee will consider peer group and other industry compensation data and the recommendations of Pearl Meyer when making decisions related to executive

compensation, with the goal of ensuring that our compensation levels are reasonably competitive relative to the compensation paid by companies in our peer group. Based in part on initial consultation with Pearl Meyer and review of Pearl Meyer s analysis and recommendations, we generally expect that our board of directors and compensation committee will, in making future compensation decisions, target the total compensation paid to our executive officers between the 50th and 75th percentile of companies in our peer group.

Annual compensation review process

We conducted annual compensation reviews for the first time in December 2011. As part of the reviews, we addressed bonus awards for 2011, our first full year of operations, and for all aspects of compensation for 2012. In future years, we expect to evaluate each executive officer s performance during the year in the first quarter of the following year. We expect that our chief executive officer will evaluate each executive other than himself from his own perspective and based on input from others within our company. This process will lead to a recommendation by the chief executive officer to the compensation committee with respect to each executive officer, other than himself, as to:

- the level of contributions made to the general management and guidance of the company;

- the need for salary increases;

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- the amount of bonuses to be paid, including the achievement of stated corporate and individual performance goals with respect to the annual review for performance in 2012 and future years; and

- whether or not equity incentive awards should be made.

These recommendations will be reviewed by our compensation committee and taken into account when it makes a final determination on all such matters.

Components of our executive compensation program

The primary elements of our executive compensation program are:

- base salary;

- annual performance-based cash bonuses;

- stock-based awards;

- broad-based health and welfare benefits; and

- severance and change in control benefits.

We do not, and do not expect in the future to, have a formal or informal policy for allocating between long-term and short-term compensation, between cash and non-cash compensation or among the different forms of non-cash compensation. Instead, our board of directors, after reviewing data it considers relevant, has determined subjectively what it believes to be the appropriate level and mix of the various compensation components. Beginning with 2012, we expect that our compensation committee also will consider information provided to it by Pearl Meyer in making this determination. Ultimately, the objective in allocating between long-term and currently paid compensation is to ensure adequate base compensation to attract and retain personnel, while providing incentives to maximize long-term value for our company and our stockholders. Therefore, we provide cash compensation in the form of base salary to meet competitive salary norms and in the form of bonus compensation to incentivize and reward superior performance on an annual basis. To further focus our executives on longer-term performance

and the creation of stockholder value, we rely upon equity-based awards that vest over a meaningful period of time. In addition, we provide our executives with benefits that are generally available to all our employees, including health and dental insurance, life and disability insurance and a 401(k) plan. Finally, we offer our executives severance benefits to incentivize them to continue to achieve stockholder value in connection with change in control or other situations in which they could be terminated without cause.

We have employment agreements with three of our named executive officers, Mr. Forrester, Dr. Pachter and Mr. Brannelly. These employment agreements provide for specific base salaries, target annual bonuses and severance and change in control arrangements for these executive officers. Dr. Pachter also received a signing bonus and reimbursement of certain relocation expenses in connection with the commencement of his employment. Details of these employment agreements are provided below under the heading Employment agreements.

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Base salary

We use base salaries to recognize the experience, skills, knowledge and responsibilities of our employees, including our executive officers. Base salaries for our named executive officers were established through arm s-length negotiation at the time the executive was hired, taking into account the position for which the executive was considered and the executive s qualifications, prior experience and prior salary. None of our named executive officers is currently party to an employment agreement that provides for automatic or scheduled increases in base salary. However, we expect that our compensation committee will annually review and evaluate, with input from our chief executive officer, the need for adjustment of the base salaries of our executives based on changes and expected changes in the scope of an executive s responsibilities, including promotions, the individual contributions made by and performance of the executive during the prior year, the executive s performance over a period of years, overall labor market conditions, the relative ease or difficulty of replacing the executive with a well-qualified person, our overall growth and development as a company, general salary trends in our industry and among our peer group and where the executive s salary falls in the salary range presented by that data. In making decisions regarding salary increases, we may also draw upon the experience of members of our board of directors with other companies. We do not expect that our executive officers will receive any formulaic base salary increase, but we do expect that our compensation committee will, in making future compensation decisions, target the total cash compensation of our named executive officers, consisting of their base salaries and target annual cash bonuses, generally between the 50th and 75th percentile of companies in our peer group.

Dr. Westphal does not currently receive, and has not historically received, a base salary from us. Dr. Westphal receives annual compensation in connection with his service on our board of directors, as further described under the heading Director compensation.

Mr. Forrester s 2011 annual base salary was $310,000 pursuant to the terms of the employment agreement that we entered into with him upon the commencement of his employment in March 2011. Dr. Pachter s 2011 annual base salary was $280,000 pursuant to the terms of the employment agreement that we entered into with him upon the commencement of his employment in July 2011. Our board of directors approved the base salaries of Mr. Forrester and Dr. Pachter based on the recommendations of Dr. Westphal. In making his recommendations, Dr. Westphal considered the factors discussed above, including the qualifications, prior experience and prior salary of each of Mr. Forrester and Dr. Pachter. Our employment agreements with Mr. Forrester and Dr. Pachter were amended and restated effective upon the closing of our IPO in February 2012.

Mr. Brannelly s 2011 base salary was $125,000 for the first eight months of 2011 when he was serving as our part-time employee and was increased to $250,000 in September 2011 when he began serving as our full-time employee. Dr. Jindal was paid $300,000 in total salary for 2011 as our former President and Chief Operating Officer and in his current capacity as our non-executive employee pursuant to the terms of a transition services agreement we entered into with him in February 2011, which provides a current annual base salary of $300,000 through mid-April 2012. Prior to his departure in August 2011, Dr. Elliott was paid $108,000 in total salary for 2011. As with our current executive officers, the base salary for each of these individuals was determined at the time of hire based on the factors set forth above.

For 2012, our board of directors determined to increase the base salaries for the named executive officers currently employed by us from 2011 levels based on our board s view, and the recommendation of Pearl Meyer, with respect to typical annual salary increases for employees in our industry. In addition, the amended and restated employment agreements effective upon the closing of our IPO in February 2012 provided for further increases in the base salaries for our current executive officers to recognize their increased responsibilities with respect to serving as executives of a publicly-traded company. Mr. Forrester s annual base salary is $370,000.

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Dr. Pachter s annual base salary is $300,000. Mr. Brannelly s base salary is $258,000. We believe that these base salaries established for our named executive officers are aligned with our executive compensation objectives stated above and are competitive with those of similarly-situated companies.

Annual performance-based cash bonus

Because we only commenced operations in November 2010, none of our named executive offices received an annual cash bonus for 2010. Our board of directors subjectively determined the amount of annual cash bonuses for our current executive officers for 2011 in December 2011. We did not establish specific corporate or individual performance goals for our executive officers for 2011.

Dr. Westphal did not receive an annual cash bonus for 2011. In accordance with the terms of their employment agreements with us, Mr. Forrester and Dr. Pachter were eligible to receive an annual bonus for 2011 based on a percentage of their base salary. Mr. Forrester has an annual bonus target of 35% of his base salary, and Dr. Pachter has an annual bonus target of 30% of his base salary. Our board of directors awarded Mr. Forrester a 2011 bonus of $130,000 and Dr. Pachter a 2011 bonus of $38,500. Our board of directors also awarded Mr. Brannelly a 2011 discretionary cash bonus of $55,000. Our board s determination of these bonus awards was based primarily on its consideration of key company achievements during 2011, including the following:

- operational achievements related to hiring our team of employees, consultants and contract research organizations and our scientific advisory board, establishing a facility consisting of office and laboratory space, raising capital through preferred stock financings and filing a registration statement for our IPO;

- product discovery achievements related to screening compounds, selecting early development candidates, establishing the putative mechanism of action of VS-507, progressing our understanding of CSC biology and focusing on key CSC-related pathways;

- product development achievements related to preclinical development of our lead product candidates;

- biomarker and diagnostic achievements related to selecting potential genetic and protein biomarkers for validation studies; and

- business development achievements related to transactions with the Whitehead Institute for Biomedical Research, the Broad Institute, the Massachusetts Institute of Technology, the President and Fellows of Harvard College and Poniard Pharmaceuticals, Inc.

Neither Dr. Jindal nor Dr. Elliott received an annual bonus for 2011.

We are in the process of designing an annual cash bonus program to reward our named executive officers in the future. We expect that our annual cash bonus program will be based upon the achievement of specified annual corporate and individual goals that will be established in advance by our compensation committee. We expect that our annual cash bonus program will emphasize pay-for-performance and will be intended to closely align executive compensation with achievement of specified operating results as the amount will be calculated on the basis of percentage of corporate goals achieved. The performance goals established by our compensation committee beginning with the 2012 fiscal year will be based on the business strategy of the company and the objective of building stockholder value. We expect that there will be three steps to determine if and the extent to

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which an annual cash bonus is payable to a named executive officer. First, at the beginning of the year, our compensation committee will determine the target annual cash incentive award for the named executive officer based on a percentage of the officer s annual base salary for that year. Second, the compensation committee will establish the specific performance goals, including both corporate and individual objectives, that must be met for the officer to receive the award. Third, shortly after the end of the year, the compensation committee will determine the extent to which these performance goals were met and the amount of the award. Our compensation committee is currently working with our chief executive officer to develop corporate and individual goals that they believe can be reasonably achieved with hard work over the course of the year and will target total cash compensation, consisting of base salaries and target annual cash bonuses, generally between the 50th and 75th percentile of companies in our peer group. The amended and restated employment agreements effective upon our IPO in February 2012 provide for increases in the target bonus percentage for our current executive officers to recognize their increased responsibilities with respect to serving as executives of a publicly-traded company. Mr. Forrester s agreement provides for an annual bonus target of 40% of his base salary and Dr. Pachter s agreement provides for an annual bonus target of 35% of his base salary. The employment agreement with Mr. Brannelly effective in March 2012 established a target bonus percentage to recognize Mr. Brannelly s increased responsibilities with respect to serving as a Vice President of Finance of a publicly-traded company. Mr. Brannelly s agreement provides for an annual bonus target of 30% of his base salary.

Stock-based awards

Our equity award program is the primary vehicle for offering long-term incentives to our executives. While we do not have any equity ownership guidelines for our executives, we believe that equity grants provide our executives with a strong link to our long-term performance, create an ownership culture and help to align the interests of our executives and our stockholders. In addition, the vesting feature of our equity awards contributes to executive retention by providing an incentive for our executives to remain in our employ during the vesting period. Prior to our IPO, our executives were eligible to participate in our 2010 equity incentive plan, and all equity awards granted in 2011 were pursuant to the 2010 equity incentive plan. Following the closing of our IPO in February 2012, our employees and executives became eligible to receive stock-based awards pursuant to our 2012 incentive plan. Under our 2012 incentive plan, executives are eligible to receive grants of stock options, restricted stock awards, restricted stock unit awards, stock appreciation rights and other stock-based equity awards at the discretion of our board of directors.

Our equity awards have typically been in the form of stock options. Because our executives profit from stock options only if our stock price increases relative to the stock option s exercise price, we believe stock options provide meaningful incentives for our executives to achieve increases in the value of our stock over time. While we currently expect to continue to use stock options as the primary form of equity awards that we grant, we have used and may in the future continue to use alternative forms of equity awards, such as restricted stock and restricted stock units.

To date, we have generally used equity awards to compensate our executive officers in the form of initial grants in connection with the commencement of employment. However, we have also approved restricted stock units, granted effective upon the closing of our IPO, to our executive officers other than Dr. Westphal as further described under the heading Grants of plan-based awards in 2011. In the future, we also generally plan to grant equity awards on an annual basis to our executive officers. We expect that, beginning in 2012, our compensation committee generally will target the equity awards of our executive officers at the 75th percentile of companies in our peer group. We may also make additional discretionary grants, typically in connection with

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the promotion of an employee, to reward an employee, for retention purposes or in other circumstances recommended by management.

In general, the equity awards that we have granted to our executives vest with respect to 25% of the shares on the first anniversary of the grant date and with respect to the remaining shares in approximately equal quarterly installments through the fourth anniversary of the grant date. Vesting ceases upon termination of employment and exercise rights cease shortly after termination of employment. Prior to the exercise of a stock option, the holder has no rights as a stockholder with respect to the shares subject to such option, including voting rights or the right to receive dividends or dividend equivalents.

We have granted stock options with exercise prices that are set at no less than the fair value of shares of our common stock on the date of grant as determined by our board of directors. The exercise price of all stock options granted after the closing of our IPO will be equal to the fair value of shares of our common stock on the date of grant, which generally will be determined by reference to the closing market price of our common stock on the date of grant.

We have not granted any equity awards to Dr. Westphal in connection with his service as our President and Chief Executive Officer. As one of our co-founders, we issued and sold to Dr. Westphal 628,571 shares of our common stock at a price per share of $0.00035 in August 2010 in connection with our formation. These shares are subject to repurchase by us pursuant to a restricted stock agreement with Dr. Westphal. These shares vest with respect to 25% of the shares on the grant date and with respect to the remaining shares in approximately equal quarterly installments through the fourth anniversary of the grant date. In addition Dr. Westphal has received an annual stock option awards in connection with his service on our board of directors, as further described under the heading Director compensation.

In April 2011, in recognition of the commencement of Mr. Forrester s employment with us, we issued and sold to Mr. Forrester 128,000 shares of our common stock pursuant to his employment agreement. These shares are subject to repurchase by us pursuant to the terms of a restricted stock agreement. These shares vest with respect to 25% of the shares on the first anniversary of his date of hire and with respect to the remaining shares in approximately equal monthly installments through the fourth anniversary of his date of hire. The purchase price of the restricted stock was $0.28 per share, the fair value of our common stock on the date of grant as determined by our board of directors. We also granted restricted stock units to Mr. Forrester, effective upon the closing of our IPO, as described under the heading Grants of plan-based awards in 2011.

In September 2011, in recognition of the commencement of Dr. Pachter s employment with us, we granted Dr. Pachter an option to purchase 68,571 shares of our common stock pursuant to his employment agreement. This option vests with respect to 25% of the shares on the first anniversary of his date of hire and with respect to the remaining shares in approximately equal quarterly installments through the fourth anniversary of his date of hire. The exercise price of this option is $1.93 per share, the fair value of our common stock on the date of grant as determined by our board of directors. We also granted restricted stock units to Dr. Pachter, effective upon the closing of our IPO, as described under the heading Grants of plan-based awards in 2011.

We did not grant any equity awards to Mr. Brannelly in 2011. In December 2010, in recognition of the commencement of Mr. Brannelly s employment with us, we granted Mr. Brannelly an option to purchase 60,000 shares of our common stock. This option vests with respect to 25% of the shares on the first anniversary of his date of hire and with respect to the remaining shares in approximately equal quarterly installments through the fourth anniversary of his date of hire. The exercise price of this option is $0.28 per share, the fair value of our common stock on the date of grant as determined by our board of directors. We also granted

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restricted stock units to Mr. Brannelly, effective upon the closing of our IPO, as described under the heading Grants of plan-based awards in 2011.

We did not grant any equity awards to Dr. Jindal in 2011. As one of our co-founders, we issued and sold to Dr. Jindal 357,142 shares of our common stock in August 2010 in connection with our formation. Pursuant to a restricted stock agreement with Dr. Jindal, as amended, we repurchased 166,480 shares from him.

In April 2011, in recognition of the commencement of Dr. Elliott s employment with us, we issued and sold to Dr. Elliott 128,000 shares of our common stock pursuant to his employment agreement at a price of $0.28 per share, the fair value of our common stock on the date of grant as determined by our board of directors. Pursuant to a restricted stock agreement with Dr. Elliott, we repurchased 120,000 shares in connection with Dr. Elliott s transition from our employee to a member of our scientific advisory board.

Benefits and other compensation

We believe that establishing competitive benefit packages for our employees is an important factor in attracting and retaining highly qualified personnel. We maintain broad-based benefits that are provided to all employees, including health and dental insurance, life and disability insurance and a 401(k) plan. All of our executives are eligible to participate in all of our employee benefit plans, in each case on the same basis as other employees. Under our 401(k) plan, we match 100% of employee contributions up to an amount equal to 3% of the employee s salary and then match 50% of employee contributions up to an amount equal to an additional 2% of the employee s salary. The match vests immediately. Consistent with our compensation philosophy, we intend to continue to maintain our current benefits for our named executive officers.

In certain circumstances, we may award cash signing bonuses or may reimburse relocation expenses when executives first join us. Whether a signing bonus is paid or relocation expenses are reimbursed, and the amount of either such benefit, is determined by our board of directors on a case-by-case basis based on the specific hiring circumstances and the recommendation of our chief executive officer.

Dr. Pachter, who joined us in June 2011, received a signing bonus of $50,000 payable upon commencement of employment. We also reimbursed Dr. Pachter for $12,926 of relocation expenses in connection with his move to our area to commence employment with us.

Severance and change in control benefits

Pursuant to employment agreements we have entered into with certain of our executives, these executives are entitled to specified benefits in the event of the termination of their employment under specified circumstances, including termination following a change in control of our company. Please refer to Employment agreements for a more detailed discussion of these benefits. We have provided estimates of the value of the severance payments made and other benefits provided to executives under various termination circumstances, under the heading Potential payments upon termination or change in control below.

We believe providing these benefits helps us compete for executive talent. After reviewing the practices of companies represented in the compensation peer group, we believe that our severance and change in control benefits are generally in line with severance packages offered to executives of the companies in our peer group. Based on the substantial business experience of the members of our board of directors and consultation with Pearl Meyer, we believe that our severance and change in control benefits are generally in line with severance

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packages offered to executives by companies at comparable stages of development in our industry and related industries.

We have structured our change in control benefits as double trigger benefits. In other words, the change in control does not itself trigger benefits. Rather, benefits are paid only if the employment of the executive is terminated during a specified period in connection with the change in control. We believe a double trigger benefit maximizes stockholder value because it prevents an unintended windfall to executives in the event of a friendly change in control, while still providing them appropriate incentives to cooperate in negotiating any change in control in which they believe they may lose their jobs.

Risk considerations in our compensation program

Our compensation committee is evaluating the philosophy and standards on which our compensation plans will be implemented across our company. It is our belief that our compensation programs do not, and in the future will not, encourage inappropriate actions or risk taking by our executive officers. We do not believe that any risks arising from our employee compensation policies and practices are reasonably likely to have a material adverse effect on our company. In addition, we do not believe that the mix and design of the components of our executive compensation program will encourage management to assume excessive risks. We believe that our current business process and planning cycle fosters the behaviors and controls that would mitigate the potential for adverse risk caused by the action of our executives. We believe that the following aspects of our executive compensation program that we plan to implement will mitigate the potential for adverse risk caused by the action of our executives:

- annual establishment of corporate and individual objectives for our performance-based cash bonus programs for our executive officers, which we expect to be consistent with our annual operating and strategic plans, designed to achieve the proper risk/reward balance and not require excessive risk taking to achieve;

- the mix between fixed and variable, annual and long-term and cash and equity compensation, which we expect to be designed to encourage strategies and actions that balance the company s short-term and long-term best interests; and

- equity incentive awards that vest over a period of time, which we believe will encourage executives to take a long-term view of our business.

Tax and accounting considerations

Section 162(m) of the Internal Revenue Code of 1986, as amended, generally disallows a tax deduction for compensation in excess of $1.0 million paid to our chief executive officer and our three other most highly paid officers (other than the chief executive officer and the chief financial officer). Qualifying performance-based compensation is not subject to the deduction limitation if specified requirements are met. We will periodically review the potential consequences of Section 162(m) and we generally intend to structure the performance-based portion of our executive compensation, where feasible, to comply with exemptions in Section 162(m) so that the compensation will remain tax deductible to

us. However, the board of directors or compensation committee may, in its judgment, authorize compensation payments that do not comply with the exemptions in Section 162(m) when it believes that such payments are appropriate to attract and retain executive talent and are in the best interests of our stockholders.

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We account for equity compensation paid to our employees in accordance with Financial Accounting Standards Board, or FASB, Accounting Standard Codification Topic 718, *Compensation-Stock Compensation*, or ASC 718, which requires us to measure and recognize compensation expense in our financial statements for all share-based payments based on an estimate of their fair value over the service period of the award. We record cash compensation as an expense at the time the obligation is accrued.

SUMMARY COMPENSATION TABLE

The following table sets forth the total compensation awarded to, earned by or paid to our named executive officers during 2011.

Name and principal position	Year	Salary ($)	Bonus ($)	Stock awards ($)(1)	Option awards ($)(2)	All other compensation ($)(3)	Total ($)
Christoph Westphal, M.D., Ph.D.(4) *President and Chief Executive Officer*	2011						
Robert Forrester *Chief Operating Officer*	2011	252,775	130,000	35,840		7,052	425,667
Jonathan Pachter, Ph.D. *Vice President, Head of Research*	2011	129,236	88,500(5)		81,336	20,440	319,512
Paul Brannelly *Vice President of Finance, Former principal financial officer*	2011	164,427	55,000			4,539	223,966
Satish Jindal, Ph.D.(6) *Former President and Chief Operating Officer*	2011	300,019				4,521	304,540
Peter Elliott, Ph.D.(7) *Former Head of Research and Development*	2011	108,505		35,840		1,728	146,073

(1) The amounts in the Stock awards column reflect the aggregate grant date fair value of restricted stock granted during the year computed in accordance with the provisions of ASC 718, excluding the impact of estimated repurchases by us related to service-based vesting conditions. The assumptions that we used to calculate these amounts are discussed in Note 6 to our financial statements appearing elsewhere in this Annual Report on Form 10-K.

(2) The amounts in the Option awards column reflect the aggregate grant date fair value of stock options granted during the year computed in accordance with the provisions of ASC 718, excluding the impact of estimated forfeitures related to service-based vesting conditions (which in our case were none). The assumptions that we used to calculate these amounts are discussed in Note 6 to our financial statements appearing elsewhere in this Annual Report on Form 10-K.

(3) The amounts in the All other compensation column reflect the value of perquisites and other personal benefits, which are further detailed below.

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Name	401(k) match ($)	Group life insurance premium ($)	Relocation expense reimbursement ($)	Total ($)
Christoph Westphal, M.D., Ph.D.				
Robert Forrester	6,677	375		7,052
Jonathan Pachter, Ph.D.	7,169	345	12,926	20,440
Paul Brannelly	4,269	270		4,539
Satish Jindal, Ph.D.	3,231	1,290		4,521
Peter Elliott, Ph.D.	1,383	345		1,728

(4) Dr. Westphal did not receive any compensation from us for his service as our President and Chief Executive Officer in 2011.

(5) The bonus amount for Dr. Pachter includes a signing bonus of $50,000 paid upon the commencement of his employment with us.

(6) In February 2011, Dr. Jindal transitioned from his former role as our President and Chief Operating Officer to his current capacity as our non-executive employee pursuant to the terms of a transition services agreement.

(7) Dr. Elliott s employment with us ended in August 2011.

GRANTS OF PLAN-BASED AWARDS IN 2011

The following table sets forth information regarding grants of plan-based awards to our named executive officers during 2011.

Name	Grant date	All other stock awards: number of shares of stock (#)	All other option awards: number of securities underlying options (#)	Exercise price of option awards ($/share)(1)	Grant date fair value of stock and option awards ($)(2)
Christoph Westphal, M.D., Ph.D.					
Robert Forrester	3/3/2011	128,000(3)			35,840
Jonathan Pachter, Ph.D.	9/6/2011		68,571(4)	1.93	81,336
Paul Brannelly					
Satish Jindal, Ph.D.					
Peter Elliott, Ph.D.	3/3/2011	128,000(5)			35,840

(1) Option awards have been granted with exercise prices equal to the fair value of our common stock on the date of grant. For a discussion of our methodology for determining the fair value of our common stock, see Management s discussion and analysis of financial condition and results of operations Critical accounting policies and significant judgments and estimates.

(2) The amounts in the Grant date fair value of stock and option awards column reflect the grant date fair value of stock and option awards granted in 2011 calculated in accordance with ASC 718.

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(3) Mr. Forrester paid $0.28 per share for the stock award. Stock award vests with respect to 25% of the shares on the first anniversary of Mr. Forrester s date of hire, which was in March 2011, and with respect to the remaining shares in approximately equal monthly installments through the fourth anniversary of his date of hire.

(4) Option award vests with respect to 25% of the shares on the first anniversary of Dr. Pachter s date of hire, which was in July 2011, and with respect to the remaining shares in approximately equal quarterly installments through the fourth anniversary of his date of hire.

(5) Dr. Elliott paid $0.28 per share for the stock award. Pursuant to a restricted stock agreement with Dr. Elliott, we repurchased 120,000 shares in connection with Dr. Elliott s transition from our employee to a member of our scientific advisory board. The remaining shares of stock are fully vested.

In December 2011 we approved awards of restricted stock units, which were granted effective upon the closing of our IPO in February 2012, to various employees, including our executive officers, as part of our effort to bring our equity compensation more into line with that of companies in our peer group. The restricted stock units approved in 2011 for our named executive officers other than Dr. Westphal are as follows:

Name	Number of restricted stock units
Robert Forrester	142,857
Jonathan Pachter, Ph.D.	85,714
Paul Brannelly	28,571

Each restricted stock unit represents the right to receive one share of our common stock if the vesting conditions are satisfied. The restricted stock units vest with respect to 25% of the shares on the first anniversary of the closing of our IPO and with respect to the remaining shares in approximately equal semi-annual installments through the fourth anniversary of the closing of our IPO.

OUTSTANDING EQUITY AWARDS AT DECEMBER 31, 2011

The following table sets forth information regarding outstanding equity awards held by our named executive officers as of December 31, 2011.

	Option awards				Stock awards	
Name	Number of securities underlying unexercised options (#) exercisable	Number of securities underlying unexercised options (#) unexercisable	Option exercise price ($)	Option expiration date	Number of shares that have not vested (#)	Market value of shares that have not vested ($)
					324,107(1)	3,241,070(2)

Christoph Westphal, M.D., Ph.D.						
Robert Forrester					128,000(3)	1,280,000(2)
Jonathan Pachter, Ph.D.		68,571(4)	1.93	9/6/2021		
Paul Brannelly	15,000	45,000(5)	0.28	12/3/2020		
Satish Jindal, Ph.D.					17,671(6)	176,710(2)
Peter Elliott, Ph.D.						

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(1) Stock award vested with respect to 25% of the shares on the grant date, which was in August 2010, and vests with respect to the remaining shares in approximately equal quarterly installments through the fourth anniversary of the grant date.

(2) The market value of the stock award is based on our IPO price of $10.00 per share.

(3) Stock award vests with respect to 25% of the shares on the first anniversary of Mr. Forrester s date of hire, which was in March 2011, and with respect to the remaining shares in approximately equal monthly installments through the fourth anniversary of his date of hire.

(4) Option award vests with respect to 25% of the shares on the first anniversary of Dr. Pachter s date of hire, which was in July 2011, and with respect to the remaining shares in approximately equal quarterly installments through the fourth anniversary of his date of hire.

(5) Option award vests with respect to 25% of the shares on the first anniversary of Mr. Brannelly s date of hire, which was in November 2010, and with respect to the remaining shares in approximately equal quarterly installments through the fourth anniversary of his date of hire.

(6) Stock award vests in installments specified in a restricted stock agreement with Dr. Jindal, as amended, and became fully vested in February 2012.

OPTIONS EXERCISED AND STOCK VESTED

None of our named executive officers exercised any options during 2011. The following table sets forth information regarding the vesting of stock during 2011 for each of our named executive officers.

	Stock awards	
Name	**Number of shares acquired on vesting (#)**	**Value realized on vesting ($)(1)**
Christoph Westphal, M.D., Ph.D.	117,857(2)	229,969
Robert Forrester		
Jonathan Pachter, Ph.D.		
Paul Brannelly		
Satish Jindal, Ph.D.	66,964(3)	130,663
Peter Elliott, Ph.D.	8,000(4)	15,400

(1) The value realized upon vesting is equal to the fair value of our common stock on the vesting date multiplied by number of shares acquired on vesting.

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(2) Stock award vested with respect to 25% of the shares on the grant date, which was in August 2010, and vests with respect to the remaining shares in approximately equal quarterly installments through the fourth anniversary of the grant date.

(3) Stock award vests in installments specified in a restricted stock agreement with Dr. Jindal, as amended, and will be fully vested in February 2012.

(4) Pursuant to a restricted stock agreement with Dr. Elliott, we repurchased 120,000 shares in connection with Dr. Elliott s transition from our employee to a member of our scientific advisory board. The remaining shares of stock are fully vested.

EMPLOYMENT AGREEMENTS

In connection with the commencement of their employment with us, we entered into employment agreements with each of Mr. Forrester and Dr. Pachter. We amended and restated these agreements effective upon the closing of our IPO in February 2012. We also entered into an employment agreement with Mr. Brannelly, a former executive officer and our current Vice President of Finance, in March 2012. Each of these employment agreements provides that employment will continue for an indefinite period until either we or the employee provides written notice of termination in accordance with the terms of the agreement. In addition, each of these executive officers is bound by the terms of an employee non-solicitation, non-competition, confidential information and inventions assignment agreement that, among other things, prevents the executive from competing with us during the term of his employment and for a specified time thereafter.

Pursuant to the terms of the amended and restated employment agreements, Mr. Forrester, Dr. Pachter and Mr. Brannelly will receive the following base salaries and will be eligible for the following bonus percentages.

Name	Annual Base Salary ($)	Bonus Percentage (%)
Robert Forrester	370,000	40
Jonathan Pachter, Ph.D.	300,000	35
Paul Brannelly	258,000	30

Upon execution and effectiveness of a release of claims, each of Mr. Forrester, Dr. Pachter and Mr. Brannelly will be entitled to severance payments if we terminate his employment without cause, as defined in the employment agreement, or Mr. Forrester, Dr. Pachter or Mr. Brannelly terminates employment with us for good reason, as defined in the employment agreement.

If Mr. Forrester s, Dr. Pachter s or Mr. Brannelly s employment terminates under these circumstances, in each case absent a change in control, as defined in the employment agreement, we will be obligated for a period of 12 months, in the case of Mr. Forrester, 9 months, in the case of Dr. Pachter, and six months in the case of Mr. Brannelly, (1) to pay such employee his base salary, (2) to provide that any equity awards granted prior to or in connection with the closing of this offering will continue vesting and (3) to the extent allowed by applicable law and the applicable plan documents, continue to provide to such employee all company employee benefit plans and arrangements that he was receiving at the time of termination.

If Mr. Forrester s, Dr. Pachter s or Mr. Brannelly s employment terminates under these circumstances, in each case within 90 days prior to, or 18 months following, a change in control, we will be obligated (1) to pay such employee a lump sum amount equal to 12 months of his base salary, in the case of Mr. Forrester and Dr.

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Pachter, and six months of his base salary, in the case of Mr. Brannelly, (2) accelerate in full the vesting of all outstanding equity awards and (3) to the extent allowed by applicable law and the applicable plan documents, continue to provide to such employee, for a period of 12 months, in the case of Mr. Forrester and Dr. Pachter, and six months, in the case of Mr. Brannelly, all company employee benefit plans and arrangements that he was receiving at the time of termination.

To the extent that any severance or compensation payment to Mr. Forrester pursuant to his employment agreement constitutes an excess parachute payment within the meaning of Sections 280G and 4999 of the Internal Revenue Code, then Mr. Forrester will be entitled to an additional gross-up payment equal to the sum of the amount of tax owed by him in connection with such excess parachute payment and any interest or penalties thereon.

POTENTIAL PAYMENTS UPON TERMINATION OR CHANGE IN CONTROL

The following tables set forth information regarding potential payments that each named executive officer who was serving as an executive officer as of December 31, 2011 would have received if the executive officer s employment had terminated as of December 31, 2011 under the circumstances set forth below, assuming that the amended and restated employment agreements described above for each of the named executive officers were in effect as of December 31, 2011.

	Termination without cause or for good reason absent a change in control		
Name	**Cash payment $**	**Value of stock-based awards with accelerated vesting (1) $**	**Value of benefits $**
Robert Forrester	370,000	544,349	2,163,127(2)
Jonathan Pachter, Ph.D.	225,000	138,422	15,210
Paul Brannelly	129,000	146,642	10,140

(1) The value of stock options with accelerated vesting represents the value of unvested stock options as of December 31, 2011 based on the difference between the exercise price of the options and the IPO price of $10.00 per share.

(2) Under the terms of the conditional 280G gross-up provisions in Mr. Forrester s amended and restated employment agreement described above, Mr. Forrester would receive an additional severance payment in the amount of $2,163,127 to ensure appropriate treatment of any excess parachute payments to Mr. Forrester within the meaning of Sections 280G and 4999 of the Internal Revenue Code.

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	Termination without cause or for good reason within 90 days prior to, or 18 months following, a change in control		
Name	Cash payment $	Value of stock-based awards with accelerated vesting(1) $	Value of benefits $
Robert Forrester	370,000	2,672,730	2,163,127(2)
Jonathan Pachter, Ph.D.	300,000	1,410,851	20,280
Paul Brannelly	129,000	585,710	10,140

(1) The value of stock options with accelerated vesting represents the value of unvested stock options as of December 31, 2011 based on the difference between the exercise price of the options and our IPO price of $10.00 per share.

(2) Under the terms of the conditional 280G gross-up provisions in Mr. Forrester s amended and restated employment agreement described above, Mr. Forrester would receive an additional severance payment in the amount of $2,163,127 to ensure appropriate treatment of any excess parachute payments to Mr. Forrester within the meaning of Sections 280G and 4999 of the Internal Revenue Code.

PENSION BENEFITS

We do not maintain any defined benefit pension plans.

NONQUALIFIED DEFERRED COMPENSATION

We do not maintain any nonqualified deferred compensation plans.

401(K) RETIREMENT PLAN

We maintain a defined contribution employee retirement plan for our employees. Our 401(k) plan is intended to qualify as a tax-qualified plan under Section 401 of the Internal Revenue Code so that contributions to our 401(k) plan, and income earned on such contributions, are not taxable to participants until withdrawn or distributed from the 401(k) plan. Our 401(k) plan provides that each participant may contribute up to 100% of his or her pre-tax compensation, up to a statutory limit, which is $17,000 for 2012. Participants who are at least 50 years old can also make catch-up contributions, which in 2012 may be up to an additional $5,500 above the statutory limit. Under our 401(k) plan, each employee is fully vested in his or her deferred salary contributions. Employee contributions are held and invested by the plan s trustee. Our 401(k) plan also permits us to make discretionary contributions and matching contributions, subject to established limits and a vesting schedule. Beginning in July 2011, we made an employer matching contribution equal to (1) 100% of employee deferral contributions up to a deferral rate of 3% of

compensation plus (2) 50% of employee deferral contributions up to a deferral rate of an additional 2% of compensation.

DIRECTOR COMPENSATION

During 2011, we did not pay cash compensation to any director for his service as a director, except Henri Termeer. Mr. Termeer received an annual retainer fee of $25,000 for his service on our board of directors in 2011. We have historically reimbursed our non-employee directors for reasonable travel and other expenses incurred in connection with attending board of director and committee meetings.

As discussed in the Executive compensation section of this Annual Report on Form 10-K, our President and Chief Executive Officer, Christoph Westphal, M.D., Ph.D., who is also chairman of our board of directors, has

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not historically received any compensation in connection with his service as our President and Chief Executive Officer. Following the closing of our IPO in February 2012, Dr. Westphal has been compensated for his service on our board of directors as described below.

During 2011, we did not grant equity awards as compensation to any of our directors, except Henri Termeer. In June 2011, in recognition of the commencement of his service on our board of directors, we granted Mr. Termeer an option to purchase 35,714 shares of our common stock. This option vests with respect to 25% of the shares on the first anniversary of the grant date and with respect to the remaining shares in approximately equal quarterly installments through the fourth anniversary of the grant date. The exercise price of this option is $0.28 per share, the fair value of our common stock on the date of grant as determined by our board of directors.

Following the closing of our IPO in February 2012, our directors were compensated, and will be compensated in the future, for service on our board of directors as follows:

- an annual retainer for our non-employee directors for service on our board of directors of $30,000;
- for members of the audit committee, an annual fee of $7,500 ($15,000 for the chair);
- for non-employee members of the nominating and corporate governance committee, an annual fee of $3,750 ($7,500 for the chair);
- for members of the compensation committee, an annual fee of $5,000 ($10,000 for the chair);
- for any non-employee chairman of our board of directors, an additional annual fee of $40,000;
- for any lead director of our board of directors, an additional annual fee of $20,000;
- for any newly elected director, an initial stock option grant of 25,000 shares of our common stock; and
- an annual stock option grant for continuing service on our board of directors of 12,500 shares of our common stock.

Subject to the director s continued service as a director, the initial and annual stock option grants will vest in approximately equal monthly installments through the first anniversary of the grant date.

In addition, we will continue to reimburse our non-employee directors for reasonable travel and other expenses incurred in connection with attending board of director and committee meetings.

COMPENSATION COMMITTEE INTERLOCKS AND INSIDER PARTICIPATION

None of our executive officers serves as a member of the board of directors or compensation committee, or other committee serving an equivalent function, of any other entity that has one or more of its executive officers serving as a member of our board of directors or our compensation committee. From December 2010 until December 2011, the members of our compensation committee were John K. Clarke, Stephen Kraus and Christoph Westphal, M.D., Ph.D. Neither Mr. Clarke nor Mr. Kraus is or has been an officer or employee of our company. Dr. Westphal has served as our President and Chief Executive Officer since September 2011. For a description of transactions between us and members of our compensation committee and affiliates of such members, please see Transactions with related persons.

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COMPENSATION COMMITTEE REPORT

The Compensation Committee has reviewed and discussed the Compensation Discussion and Analysis required by Item 402(b) of Regulation S-K with our management. Based on this review and discussion, the Compensation Committee recommended to our board of directors that the Compensation Discussion and Analysis be included in this Annual Report on Form 10-K.

By the Compensation Committee of the Board of Directors,
Henri Termeer (chair)
Richard Aldrich
Stephen Kraus

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Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters

Securities Authorized for Issuance Under Equity Compensation Plans

See Securities Authorized for Issuance Under Equity Compensation Plans in Item 5 of this Annual Report on Form 10-K.

Security Ownership of Certain Beneficial Owners and Management

The following table sets forth information with respect to the beneficial ownership of our common stock as of March 15, 2012 by:

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

The column entitled Percentage of shares beneficially owned is based on a total of 21,059,116 shares of our common stock outstanding as of March 15, 2012.

Beneficial ownership is determined in accordance with the rules and regulations of the SEC and includes voting or investment power with respect to our common stock. Shares of our common stock subject to options that are currently exercisable or exercisable within 60 days of March 15, 2012 are considered outstanding and beneficially owned by the person holding the options for the purpose of calculating the percentage ownership of that person but not for the purpose of calculating the percentage ownership of any other person. Except as otherwise noted, we believe the persons and entities in this table have sole voting and investing power with respect to all of the shares of our common stock beneficially owned by them, subject to community property laws, where applicable. Except as otherwise set forth below, the address of the beneficial owner is c/o Verastem, Inc., 215 First Street, Suite 440, Cambridge, Massachusetts 02142.

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Name and address of beneficial owner	Number of shares beneficially owned	Percentage of shares beneficially owned
5% stockholders:		
Entities affiliated with Bessemer Venture Partners(1) 196 Broadway, 2nd Floor Cambridge, MA 02139	1,995,237	9.5%
CHP III, L.P.(2) 230 Nassau Street Princeton, NJ 08542	2,234,126	10.6%
Eastern Capital Limited(3) c/o Foreshore Corporate Services Ltd. 4th Floor, Queensgate House 113 South Church Street George Town, Grand Cayman KY1-1104 Cayman Islands	1,142,857	5.4%
Longwood Fund, LP(4) 800 Boylston Street, Suite 1555 Boston, MA 02199	2,869,841	13.6%
MPM Bioventures V, LP(5) c/o MPM Asset Management 200 Clarendon Street, 54th Floor Boston, MA 02116	2,029,593	9.6%
Directors and Executive Officers		
Christoph Westphal, M.D., Ph.D.(6)	3,500,495	16.6%
Robert Forrester	128,000	*
Jonathan Pachter, Ph.D.		
Satish Jindal, Ph.D.	190,662	*
Paul Brannelly(7)	22,500	*
Peter Elliott, Ph.D.	8,000	*
Richard Aldrich(8)	3,414,780	16.2%
John K. Clarke(9)	2,236,209	10.6%
Ansbert Gadicke, M.D.(10)	2,031,676	9.6%
Stephen Kraus(11)	2,083	*
Henri Termeer(12)	2,083	*
All executive officers and directors as a group (8 persons)	11,804,741	56.0%

* Represents beneficial ownership of less than one percent of our outstanding common stock.

(1) Consists of (a) 279,333 shares of common stock held by Bessemer Venture Partners VII Institutional L.P. (BVP Institutional), (b) 638,476 shares of common stock held by Bessemer Venture Partners VII L.P. (BVP VII), and (c) 1,077,428 shares of common stock held by BVP VII Special Opportunity Fund L.P. (BVP Special Opportunity and together with BVP Institutional and BVP VII, Bessemer Venture Partner Entities). Deer VII & Co. L.P. (Deer L.P.) is the general partner of the Bessemer Venture Partner Entities. Deer VII & Co. Ltd. is the general partner of Deer L.P. J. Edmund Colloton, Robin S. Chandra, David J. Cowan, Robert P. Goodman, Jeremy S. Levine and Robert M. Stavis are the directors of Deer VII & Co. Ltd. and share voting and dispositive power over the shares of stock held by the Bessemer Venture Partner Entities. Each of Mr. Colloton, Mr. Chandra, Mr. Cowan, Mr. Goodman, Mr. Levine and Mr. Stavis disclaims beneficial ownership of the shares identified in this footnote except as to his or her respective proportionate pecuniary interest in such shares.

(2) Consists of 2,234,126 shares of common stock. John K. Clarke, Brandon H. Hull, Charles G. Hadley and John J. Park are the managing members of CHP III Management, LLC, the General Partner of CHP III, L.P., and exercise shared voting, investment, and dispositive rights with respect to the shares of stock held by CHP III, L.P. Each of Messrs. Clarke, Hull, Hadley and Park disclaims beneficial ownership of the shares identified in this footnote except as to his respective proportionate pecuniary interest in such shares.

(3) Consists of 1,142,857 shares of common stock. Eastern Capital Limited is a direct wholly owned subsidiary of Portfolio Services Ltd., a Cayman Islands company. Kenneth Dart is the beneficial owner

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of all of the outstanding shares of Portfolio Services Ltd., which in turn owns all the outstanding shares of Eastern Capital Limited. Eastern Capital Limited and Mr. Dart have shared voting and dispositive power with respect to the shares held.

(4) Consists of 2,869,841 shares of common stock. Longwood Fund GP, LLC (the General Partner) is the general partner of Longwood Fund, LP and exercises voting and investment power with respect to securities owned directly by Longwood Fund, LP. Richard Aldrich, Michelle Dipp and Christoph Westphal are the managers of the General Partner and share voting and dispositive power with respect to the securities held by Longwood Fund, LP. The General Partner disclaims beneficial ownership of the securities owned directly by Longwood Fund, LP and this report shall not be deemed an admission that the General Partner is the beneficial owner of such securities, except to the extent of its pecuniary interest therein.

(5) Consists of 2,029,593 shares of common stock. MPM Bioventures V GP, LLC (MPM V GP) is the general partner of MPM Bioventures V, LP and MPM Bioventures V LLC (MPM V LLC) is the managing member of MPM V GP. Luke Evnin, Todd Foley, Ansbert Gadicke, Vaughn Kalian, James Scopa, Steven St. Peter and John Vander Vort are the members of MPM V LLC and have shared power to vote, hold and dispose of the shares held by MPM Bioventures V, LP. Each disclaims beneficial ownership of the securities reported herein except to the extent of his respective pecuniary interest therein.

(6) Consists of (a) 502,857 shares of common stock held by Dr. Westphal, (b) 125,714 shares of common stock held by The Fountain Irrevocable Trust of 2010, (c) 2,083 shares of common stock issuable upon exercise of stock options within 60 days of March 15, 2012 and (d) 2,869,841 shares of common stock held by Longwood Fund, LP. The trustee of The Fountain Irrevocable Trust of 2010 is James Kittler and he exercises sole voting and investment power of the shares of record held by the trust. The ultimate general partner of Longwood Fund, LP is Longwood Fund GP, LLC. Voting and investment power with respect to the shares held by Longwood Fund, LP are vested in Richard Aldrich, Michelle Dipp and Dr. Westphal, the managers of Longwood Fund GP, LLC.

(7) Consists of shares of common stock issuable upon exercise of stock options within 60 days of March 15, 2012.

(8) Consists of (a) 407,142 shares of common stock held by Mr. Aldrich, (b) 135,714 shares of common stock held by Richard H. Aldrich Irrevocable Trust of 2011, (c) 2,083 shares of common stock issuable upon exercise of stock options within 60 days of March 15, 2012 and (d) 2,869,841 shares of common stock held by Longwood Fund, LP. The trustee of the Richard H. Aldrich Irrevocable Trust of 2011 is Nicole Aldrich and she exercises sole voting and investment power over the shares of record held by the trust. The ultimate general partner of Longwood Fund, LP is Longwood Fund GP, LLC. Voting and investment power with respect to the shares held by Longwood Fund, LP. are vested in Mr. Aldrich, Michelle Dipp and Christoph Westphal, the managers of Longwood Fund GP, LLC.

(9) Consists of 2,234,126 shares of common stock held by CHP III, L.P. and 2,083 shares of common stock issuable upon exercise of stock options within 60 days of March 15, 2012. John K. Clarke, Brandon H. Hull, Charles G. Hadley and John J. Park are the managing members of CHP III Management, LLC, the General Partner of CHP III, L.P., and exercise shared voting, investment, and dispositive rights with respect to the shares of stock held by CHP III, L.P. Each of Messrs. Clarke, Hull, Hadley and Park disclaims beneficial ownership of the shares identified in this footnote except as to his respective proportionate pecuniary interest in such shares.

(10) Consists of 2,029,593 shares of common stock held by MPM Bioventures V, LP and 2,083 shares of common stock issuable upon exercise of stock options within 60 days of March 15, 2012. MPM V GP is the general partner of MPM Bioventures V, LP and MPM V LLC is the managing member of MPM V GP.

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Luke Evnin, Todd Foley, Ansbert Gadicke, Vaughn Kalian, James Scopa, Steven St. Peter and John Vander Vort are the members of MPM V LLC and have shared power to vote, hold and dispose of the shares held by MPM Bioventures V, LP. Each disclaims beneficial ownership of the securities reported herein except to the extent of his respective pecuniary interest therein.

(11) Consists of shares of common stock issuable upon exercise of stock options within 60 days of March 15, 2012. Mr. Kraus serves as an employee of Bessemer Venture Partners, the management company affiliate of the Bessemer Venture Partner Entities that hold an aggregate of 1,995,237 shares of our common stock as described above. Mr. Kraus has no voting or dispositive power with respect to the shares held by the Bessemer Venture Partner Entities and disclaims beneficial ownership of such shares except to the extent of his pecuniary interest therein.

(12) Consists of shares of common stock issuable upon exercise of stock options within 60 days of March 15, 2012.

Item 13. Certain Relationships and Related Transactions, and Director Independence

Transactions with related persons

Since January 1, 2011, we have engaged in the following transactions with our directors, executive officers, holders of more than 5% of our voting securities, and affiliates or immediately family members of our directors, executive officers and holders of more than 5% of our voting securities, and our co-founders. We believe that all of these transactions were on terms as favorable as could have been obtained from unrelated third parties.

PARTICIPATION IN INITIAL PUBLIC OFFERING

In February 2012, we issued and sold an aggregate of 6,325,000 shares of our common stock in our initial public offering at a price per share of $10.00 for an aggregate purchase price of $63.3 million. UBS Securities and Leerink Swann LLC acted as joint book-running managers of the offering and as representatives of the underwriters. The following table sets forth the number of shares of our common stock that were purchased by our 5% stockholders and their affiliates.

Name(1)	Shares of common stock
Bessemer Venture Partners(2)	100,000(3)
CHP III, L.P.(4)	250,000
Longwood Fund, LP(5)	600,000
MPM Bioventures V, LP(6)	100,000

(1) See Security Ownership of Certain Beneficial Owners and Management in Item 12 of this Annual Report on Form 10-K for more information about shares held by these entities.

(2) Stephen Kraus, a member of our board of directors, is a vice president of Bessemer Venture Partners.

(3) Consists of (a) 14,000 shares of common stock purchased by Bessemer Venture Partners VII Institutional L.P., (b) 32,000 shares of common stock purchased by Bessemer Venture Partners VII L.P. and (c) 54,000 shares of common stock purchased by BVP VII Special Opportunity Fund L.P.

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(4) John K. Clarke, a member of our board of directors, is the managing general partner of Cardinal Partners, the general partner of CHP III, L.P.

(5) Christoph Westphal, M.D., Ph.D. and Richard Aldrich, members of our board of directors, are partners of Longwood Fund, LP.

(6) Ansbert Gadicke, M.D., a member of our board of directors, is the managing director of MPM Capital and a member of MPM Bioventures V LLC, the general partner of MPM Bioventures V GP, LLC, which is the general partner of MPM Bioventures V, LP.

SERIES C PREFERRED STOCK FINANCING

In November 2011, we issued and sold an aggregate of 9,067,825 shares of our series C preferred stock at a price per share of $2.25 for an aggregate purchase price of $20.4 million. The following table sets forth the number of shares of our series C preferred stock that we issued to our 5% stockholders and their affiliates.

Name(1)	Shares of series C preferred stock
Advanced Technology Ventures VIII, L.P.	100,000
Entities affiliated with Bessemer Venture Partners(2)	133,333(3)
CHP III, L.P.(4)	444,444
Eastern Capital Limited	4,000,000
Longwood Fund, LP(5)	444,444
MPM Bioventures V, LP(6)	266,666

(1) See Security Ownership of Certain Beneficial Owners and Management in item 12 of this Annual Report on Form 10-K for more information about shares held by these entities.

(2) Stephen Kraus, a member of our board of directors, is employed by Bessemer Venture Partners and has no voting or dispositive power with respect to the shares held by entities affiliated with Bessemer Venture Partners.

(3) Consists of (a) 18,667 shares purchased by Bessemer Venture Partners VII Institutional L.P., (b) 42,667 shares purchased by Bessemer Venture Partners VII L.P. and (c) 71,999 shares purchased by BVP VII Special Opportunity Fund L.P.

(4) John K. Clarke, a member of our board of directors, is a managing member of CHP III Management, LLC, the general partner of CHP III, L.P.

(5) Christoph Westphal, M.D., Ph.D. and Richard Aldrich, members of our board of directors, are partners of Longwood Fund, LP.

(6) Ansbert Gadicke, M.D., a member of our board of directors, is the managing director of MPM Capital and a member of MPM Bioventures V LLC, the general partner of MPM Bioventures V GP, LLC, which is the general partner of MPM Bioventures V, LP.

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SERIES B PREFERRED STOCK FINANCING

In July 2011, we issued and sold an aggregate of 16,025,000 shares of our series B preferred stock at a price per share of $2.00 for an aggregate purchase price of $32,050,000. The following table sets forth the number of shares of our series B preferred stock that we issued to our 5% stockholders and their affiliates.

Name(1)	Shares of series B preferred stock
Advanced Technology Ventures VIII, L.P.	2,500,000
Entities affiliated with Bessemer Venture Partners(2)	2,500,000(3)
CHP III, L.P.(4)	2,500,000
Longwood Fund, LP(5)	3,500,000
MPM Bioventures V, LP(6)	2,500,000

(1) See Security Ownership of Certain Beneficial Owners and Management in item 12 of this Annual Report on Form 10-K for more information about shares held by these entities.

(2) Stephen Kraus, a member of our board of directors, is employed by Bessemer Venture Partners and has no voting or dispositive power with respect to the shares held by entities affiliated with Bessemer Venture Partners.

(3) Consists of (a) 350,000 shares purchased by Bessemer Venture Partners VII Institutional L.P., (b) 800,000 shares purchased by Bessemer Venture Partners VII L.P. and (c) 1,350,000 shares purchased by BVP VII Special Opportunity Fund L.P.

(4) John K. Clarke, a member of our board of directors, is a managing member of CHP III Management, LLC, the general partner of CHP III, L.P.

(5) Christoph Westphal, M.D., Ph.D. and Richard Aldrich, members of our board of directors, are partners of Longwood Fund, LP.

(6) Ansbert Gadicke, M.D., a member of our board of directors, is the managing director of MPM Capital and a member of MPM Bioventures V LLC, the general partner of MPM Bioventures V GP, LLC, which is the general partner of MPM Bioventures V, LP.

SERIES A PREFERRED STOCK FINANCING

In November 2010 and April 2011, we issued and sold an aggregate of 16,000,000 shares of our series A preferred stock at a price per share of $1.00 for an aggregate purchase price of $16,000,000. The following table sets forth the number of shares of our series A preferred stock that we issued to our 5% stockholders and their affiliates.

Name(1)	Shares of series A preferred stock
Entities affiliated with Bessemer Venture Partners(2)	4,000,000(3)
CHP III, L.P.(4)	4,000,000
Longwood Fund, LP(5)	4,000,000
MPM Bioventures V, LP(6)	4,000,000

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(1) See Security Ownership of Certain Beneficial Owners and Management in item 12 of this Annual Report on Form 10-K for more information about shares held by these entities.

(2) Stephen Kraus, a member of our board of directors, is employed by Bessemer Venture Partners and has no voting or dispositive power with respect to the shares held by entities affiliated with Bessemer Venture Partners.

(3) Consists of (a) 560,000 shares purchased by Bessemer Venture Partners VII Institutional L.P., (b) 1,280,000 shares purchased by Bessemer Venture Partners VII L.P. and (c) 2,160,000 shares purchased by BVP VII Special Opportunity Fund L.P.

(4) John K. Clarke, a member of our board of directors, is a managing member of CHP III Management, LLC, the general partner of CHP III, L.P.

(5) Christoph Westphal, M.D., Ph.D. and Richard Aldrich, members of our board of directors, are partners of Longwood Fund, LP.

(6) Ansbert Gadicke, M.D., a member of our board of directors, is the managing director of MPM Capital and a member of MPM Bioventures V LLC, the general partner of MPM Bioventures V GP, LLC, which is the general partner of MPM Bioventures V, LP.

REGISTRATION RIGHTS

We are a party to an investor rights agreement with certain holders of our common stock, including some of our directors, executive officers and 5% stockholders and their affiliates and entities affiliated with our directors. The investor rights agreement provides these holders the right, following the completion of this offering, to demand that we file a registration statement or request that their shares be covered by a registration statement that we are otherwise filing.

INDEMNIFICATION AGREEMENTS

Our certificate of incorporation provides that we will indemnify our directors and officers to the fullest extent permitted by Delaware law. In addition, we have entered into indemnification agreements with our directors.

POLICIES AND PROCEDURES FOR RELATED PERSON TRANSACTIONS

Our board of directors has adopted written policies and procedures for the review of any transaction, arrangement or relationship in which Verastem is a participant, the amount involved exceeds $120,000 and one of our executive officers, directors, director nominees or 5% stockholders, or their immediate family members, each of whom we refer to as a related person, has a direct or indirect material interest.

If a related person proposes to enter into such a transaction, arrangement or relationship, which we refer to as a related person transaction, the related person must report the proposed related person transaction to our principal financial officer. The policy calls for the proposed related person transaction to be reviewed and, if deemed appropriate, approved by our audit committee. Whenever practicable, the reporting, review and approval will occur prior to entry into the transaction. If advance review and approval is not practicable, the committee will review, and, in its discretion, may ratify the related person transaction. The policy also permits the chairman of the committee to review and, if deemed appropriate, approve proposed related person

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transactions that arise between committee meetings, subject to ratification by the committee at its next meeting. Any related person transactions that are ongoing in nature will be reviewed annually.

A related person transaction reviewed under the policy will be considered approved or ratified if it is authorized by the committee after full disclosure of the related person s interest in the transaction. As appropriate for the circumstances, the committee will review and consider:

- the related person s interest in the related person transaction;
- the approximate dollar value of the amount involved in the related person transaction;
- the approximate dollar value of the amount of the related person s interest in the transaction without regard to the amount of any profit or loss;
- whether the transaction was undertaken in the ordinary course of our business;
- whether the terms of the transaction are no less favorable to us than terms that could have been reached with an unrelated third party;
- the purpose of, and the potential benefits to us of, the transaction; and
- any other information regarding the related person transaction or the related person in the context of the proposed transaction that would be material to investors in light of the circumstances of the particular transaction.

The committee may approve or ratify the transaction only if the committee determines that, under all of the circumstances, the transaction is in our best interests. The committee may impose any conditions on the related person transaction that it deems appropriate.

In addition to the transactions that are excluded by the instructions to the SEC s related person transaction disclosure rule, our board of directors has determined that the following transactions do not create a material direct or indirect interest on behalf of related persons and, therefore, are not related person transactions for purposes of this policy:

- interests arising solely from the related person s position as an executive officer of another entity (whether or not the person is also a director of such entity) that is a participant in the transaction, where (a) the related person and all other related persons own in the aggregate less than a 10% equity interest in such entity, (b) the related person and his or her immediate family members are not involved in the negotiation of the terms of the transaction and do not receive any special benefits as a result of the transaction and (c) the amount involved in the transaction is less than the greater of $200,000 or 5% of the annual gross revenues of the company receiving payment under the transaction; and

- a transaction that is specifically contemplated by provisions of our charter or bylaws.

The policy provides that transactions involving compensation of executive officers shall be reviewed and approved by the compensation committee in the manner specified in its charter.

We did not have a written policy regarding the review and approval of related person transactions prior to this offering. Nevertheless, with respect to such transactions, it was our policy for our board of directors to consider

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the nature of and business reason for such transactions, how the terms of such transactions compared to those which might be obtained from unaffiliated third parties and whether such transactions were otherwise fair to and in the best interests of, or not contrary to, our best interests. In addition, all related person transactions required prior approval, or later ratification, by our board of directors.

Director Independence

Our board of directors has determined that all of our directors, other than Dr. Westphal, are independent directors, as defined by applicable NASDAQ Marketplace Rules. In making such determination, the board of directors considered the relationships that each such non-employee director has with our company and all other facts and circumstances that the board of directors deemed relevant in determining their independence, including the beneficial ownership of our capital stock by each non-employee director.

Item 14. Principal Accountant Fees and Services

Auditors Fees

The following table summarizes the fees of Ernst & Young LLP, our registered public accounting firm, billed to us for each of the last two fiscal years.

Fee category	2010	2011
Audit Fees (1)	$ 20,071	$ 520,601
Total Fees	20,071	520,601

(1) Audit fees consist of fees for the audit of our financial statements, the review of the interim financial statements and services associated with our registration statement on Form S-1.

All such accountant services and fees were pre-approved by our audit committee in accordance with the Pre-Approval Policies and Procedures described below.

Pre-Approval Policies and Procedures

Our Audit Committee has adopted policies and procedures relating to the approval of all audit and non-audit services that are to be performed by our registered public accounting firm. This policy generally provides that we will not engage our registered public accounting firm to render audit or non-audit services unless the service is specifically approved in advance by our Audit Committee or the engagement is entered into

pursuant to one of the pre-approval procedures described below.

From time to time, our Audit Committee may pre-approve specified types of services that are expected to be provided to us by our registered public accounting firm during the next 12 months. Any such pre-approval is detailed as to the particular service or type of services to be provided and is also generally subject to a maximum dollar amount.

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PART IV

Item 15. Exhibits and Financial Statement Schedules

Financial Statements

The following financial statements and supplementary data are filed as a part of this Annual Report on Form 10-K.

Report of Independent Registered Public Accounting Firm

Balance Sheets at December 31, 2011 and 2010

Statements of Operations for the year ended December 31, 2011, the period from August 4, 2010 (inception) to December 31, 2010 and the period from August 4, 2010 (inception) to December 31, 2011

Statements of Redeemable Convertible Preferred Stock and Stockholders Deficit for the year ended December 31, 2011, the period from August 4, 2010 (inception) to December 31, 2010 and the period from August 4, 2010 (inception) to December 31, 2011

Statements of Cash Flows for the year ended December 31, 2011, the period from August 4, 2010 (inception) to December 31, 2010 and the period from August 4, 2010 (inception) to December 31, 2011

Notes to Financial Statements

Financial Statement Schedules

All financial statement schedules are omitted because they are not applicable or the required information is included in the financial statements or notes thereto.

Exhibits

Those exhibits required to be filed by Item 601 of Regulation S-K are listed in the Exhibit Index immediately preceding the exhibits hereto and such listing is incorporated herein by reference.

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SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) 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.

By: /s/ Christoph Westphal, M.D., Ph.D.

Christoph Westphal, M.D., Ph.D.
President and Chief Executive Officer

Pursuant to the requirements of the Securities Act of 1934, this report has been signed by the following persons on behalf of the registrant in the capacities and on the dates indicated.

Signature	Title	Date
/s/ Christoph Westphal, M.D., Ph.D Christoph Westphal, M.D., Ph.D.	President, Chief Executive Officer and Director (Principal executive officer)	March 30, 2012
/s/ Robert Forrester Robert Forrester	Chief Operating Officer (Principal financial and accounting officer)	March 30, 2012
/s/ Richard Aldrich Richard Aldrich	Director	March 30, 2012
/s/ John K. Clarke John K. Clarke	Director	March 30, 2012
/s/ Ansbert Gadicke, M.D Ansbert Gadicke, M.D	Director	March 30, 2012
/s/ Stephen Kraus Stephen Kraus	Director	March 30, 2012
/s/ Henri Termeer Henri Termeer	Director	March 30, 2012

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Verastem, Inc.

(A development stage company)

FINANCIAL STATEMENTS

Year ended December 31, 2011, the period from August 4, 2010 (inception) to December 31, 2010 and the period from August 4, 2010 (inception) to December 31, 2011

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Verastem, Inc.

(A development stage company)

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

The Board of Directors and Stockholders of

Verastem, Inc.

We have audited the accompanying balance sheets of Verastem, Inc. (a development stage company) (the Company) as of December 31, 2011 and 2010, and the related statements of operations, redeemable convertible preferred stock and stockholders deficit and cash flows for the year ended December 31, 2011, the period from August 4, 2010 (inception) to December 31, 2010 and for period from August 4, 2010 (inception) to December 31, 2011. These financial statements are the responsibility of the Company s management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audits to obtain reasonable assurance about whether the financial statements are free of material misstatement. We were not engaged to perform an audit of the Company s internal control over financial reporting. Our audits included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company s internal control over financial reporting. Accordingly, we express no such opinion. An audit also includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of Verastem, Inc. as of December 31, 2011 and 2010 and the results of its operations and its cash flows for the year ended December 31, 2011, the period from August 4, 2010 (inception) to December 31, 2010 and the period from August 4, 2010 (inception) to December 31, 2011, in conformity with U.S. generally accounting principles.

/s/ Ernst & Young LLP

Boston, Massachusetts
March 30, 2012

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Verastem, Inc.

(A development stage company)

BALANCE SHEETS

(in thousands, except per share amounts)

	December 31,	
	2011	**2010**
Assets		
Current assets:		
Cash and cash equivalents	$ 20,954	$ 3,584
Short-term investments	26,857	
Prepaid expenses and other current assets	130	12
Total current assets	47,941	3,596
Property and equipment, net	709	8
Long-term investments	8,994	
Other assets	1,307	
Restricted cash	86	
Total assets	$ 59,037	$ 3,604
Liabilities, redeemable convertible preferred stock and stockholders deficit		
Current liabilities:		
Accounts payable	$ 2,273	$ 279
Accrued expenses	873	89
Total current liabilities	3,146	368
Deferred rent	74	
Liability for shares subject to repurchase	36	
Obligation to issue warrant	406	
Commitments and contingencies (*Note 8*)		
Series A redeemable convertible preferred stock, $0.0001 par value; 16,000 shares authorized, 16,000 and 4,000 shares issued and outstanding at December 31, 2011 and 2010, respectively (Liquidation preference of $16,000 as of December 31, 2011)	15,939	3,923
Series B redeemable convertible preferred stock, $0.0001 par value; 16,025 shares authorized, issued and outstanding at December 31, 2011 (Liquidation preference of $32,050 as of December 31, 2011)	31,948	
Series C redeemable convertible preferred stock, $0.0001 par value; 9,068 shares authorized, issued and outstanding at December 31, 2011 (Liquidation preference of $20,403 as of December 31, 2011)	20,254	
Common stock, $0.0001 par value; 53,093 and 30,000, shares authorized at December 31, 2011 and 2010 , respectively, 1,559 and 1,015 shares issued and outstanding at December 31, 2011 and 2010, respectively	1	1
Additional paid-in capital	1,702	96
Accumulated other comprehensive loss	(2)	
Deficit accumulated during the development stage	(14,467)	(784)
Total stockholders deficit	(12,766)	(687)
Total liabilities, redeemable convertible preferred stock and stockholders deficit	$ 59,037	$ 3,604

See accompanying notes.

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Verastem, Inc.

(A development stage company)

STATEMENTS OF OPERATIONS

(in thousands, except per share amounts)

	Year ended December 31, 2011	Period from August 4, 2010 (inception) to December 31, 2010	Period from August 4, 2010 (inception) to December 31, 2011
Operating expenses:			
Research and development	$ 9,883	$ 400	$ 10,283
General and administrative	3,815	384	4,199
Total operating expenses	13,698	784	14,482
Loss from operations	(13,698)	(784)	(14,482)
Interest income	15		15
Net loss	(13,683)	(784)	(14,467)
Accretion of preferred stock	(32)	(2)	(34)
Net loss applicable to common stockholders	$ (13,715)	$ (786)	$ (14,501)
Net loss per share applicable to common stockholders basic and diluted	$ (10.59)	$ (0.91)	$ (12.39)
Weighted-average number of common shares used in net loss per share applicable to common stockholders basic and diluted	1,295	850	1,171

See accompanying notes.

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Verastem, Inc.

(A development stage company)

STATEMENTS OF REDEEMABLE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS DEFICIT

(in thousands, except share data)

	Series A redeemable convertible preferred stock		Series B redeemable convertible preferred stock		Series C redeemable convertible preferred stock		Common stock		Additional paid-in capital	Accumulated other comprehensive loss	Deficit accumulated during the development stage	Totals stockholders deficit
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount				
Balance at August 4, 2010 (inception)		$		$		$		$	$	$	$	$
Sale of common stock to founders							714,286	1				1
Vesting of restricted stock							133,926					
Issuance of common stock in exchange for license							166,664		46			46
Issuance of Series A redeemable convertible preferred stock, net of offering costs of $79	4,000,000	3,921										
Accretion of redeemable convertible preferred stock to redemption value		2							(2)			(2)
Stock-based compensation expense									52			52
Net loss											(784)	(784)
Balance at December 31, 2010	4,000,000	3,923					1,014,876	1	96		(784)	(687)
Net loss											(13,683)	(13,683)
Unrealized loss on investments										(2)		(2)
Comprehensive loss												$ (13,685)
Issuance of Series A redeemable convertible	12,000,000	12,000										

preferred stock															
Issuance of Series B redeemable convertible preferred stock, net of offering costs of $113			16,025,000	31,937											
Issuance of Series C redeemable convertible preferred stock, net of offering costs of $153					9,067,825	20,249									
Accretion of redeemable convertible preferred stock to redemption value		16		11		5				(32)				(32)	
Vesting of restricted stock							543,712			3				3	
Stock-based compensation expense										1,635				1,635	
Balance at December 31, 2011	16,000,000	$ 15,939	16,025,000	$ 31,948	9,067,825	$ 20,254	1,558,588	$ 1	$ 1,702	$ (2)	$ (14,467)	$ (12,766)			

See accompanying notes.

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Verastem, Inc.

(A development stage company)

STATEMENTS OF CASH FLOWS

(in thousands)

	Year ended December 31, 2011	Period from August 4, 2010 (inception) to December 31, 2010	Period from August 4, 2010 (inception) to December 31, 2011
Operating activities			
Net loss	$ (13,683)	$ (784)	$ (14,467)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	83		83
Stock-based compensation expense	1,635	52	1,687
Common stock issued in exchange for license		46	46
Obligation to issue a warrant in exchange for license	439		439
Change in fair value of obligation to issue warrant	(33)		(33)
Changes in operating assets and liabilities:			
Prepaid expenses and other current assets	(118)	(12)	(130)
Other assets	(1,307)		(1,307)
Accounts payable	1,994	279	2,273
Accrued expenses and deferred rent	858	89	947
Net cash used in operating activities	(10,132)	(330)	(10,462)
Investing activities			
Purchases of property and equipment	(785)	(8)	(793)
Purchases of investments	(35,851)		(35,851)
Increase in restricted cash	(86)		(86)
Net cash used in investing activities	(36,722)	(8)	(36,730)
Financing activities			
Proceeds from issuance of redeemable convertible preferred stock	64,186	3,921	68,107
Net proceeds from the issuance of common stock	38	1	39
Net cash provided by financing activities	64,224	3,922	68,146
Increase in cash and cash equivalents	17,370	3,584	$ 20,954
Cash and cash equivalents at beginning of period	3,584		
Cash and cash equivalents at end of period	$ 20,954	$ 3,584	$ 20,954
Supplemental disclosure of non-cash financing activity			
Accretion of redeemable convertible preferred stock to redemption value	$ 32	$ 2	$ 34

See accompanying notes.

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Verastem, Inc.

(A development stage company)

NOTES TO FINANCIAL STATEMENTS

1. Organization and basis of presentation

Verastem, Inc. (the Company), incorporated on August 4, 2010 as a Delaware corporation, is a biopharmaceutical company focused on discovering and developing proprietary small molecule drugs targeting cancer stem cells along with proprietary companion diagnostics. The Company s operations to date have been limited to organizing and staffing the Company, business planning, raising capital, acquiring and developing its technology, identifying potential product candidates and undertaking preclinical studies of its most advanced product candidates. The Company has not commenced its planned principal operations. Accordingly, the Company is considered to be in the development stage as defined in Financial Accounting Standards Board Accounting Standards Codification Topic 915, *Development Stage Entities*.

The Company is subject to a number of risks similar to other life science companies in the development stage, including, but not limited to, the need to obtain adequate additional funding, possible failure of preclinical testing or clinical trials, inability to obtain marketing approval of product candidates, competitors developing new technological innovations, market acceptance of the Company s products and protection of proprietary technology. If the Company does not successfully commercialize any of its product candidates, it will be unable to generate product revenue or achieve profitability. As of December 31, 2011, the Company had a deficit accumulated during the development stage of $14.5 million. The Company expects to continue to incur operating losses in future periods. The Company had cash, cash equivalents and investments of $56.8 million as of December 31, 2011. The Company believes that the net proceeds from its initial public offering completed in February 2012, together with its existing cash, cash equivalents and investments, will be sufficient to fund its current operating plan and capital expenditure requirements for the next several years.

2. Significant accounting policies

Use of estimates

The preparation of the Company s financial statements in conformity with U.S. generally accepted accounting principles requires management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. Actual results could differ from such estimates.

Prior to becoming a public company in February 2012, the Company utilized significant estimates and assumptions in determining the fair value of its common stock. The Company granted stock options at exercise prices not less than the fair market value of its common stock as determined by the board of directors, with input from management. The board of directors determined the estimated fair value of the Company s common stock based on a number of objective and subjective factors, including external market conditions affecting the biotechnology industry

sector and the prices at which the Company sold shares of redeemable

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convertible preferred stock, the superior rights and preferences of securities senior to the Company s common stock at the time and the likelihood of achieving a liquidity event, such as an IPO or sale of the Company.

The Company utilized various valuation methodologies in accordance with the framework of the American Institute of Certified Public Accountants Technical Practice Aid, *Valuation of Privately-Held Company Equity Securities Issued as Compensation*, to estimate the fair value of its common stock. The methodologies included an asset-based approach and the current value method for the Company s initial common stock valuation as of November 30, 2010, the option pricing method utilizing the reverse backsolve method to estimate the Company s underlying equity value as of July 31, 2011 and a methodology that determined an estimated value under an IPO scenario and a sale scenario based upon an assessment of the probability of occurrence of each scenario as of September 30, 2011, November 17, 2011, and December 31, 2011. Each valuation methodology includes estimates and assumptions that require the Company s judgment. These estimates include assumptions regarding future performance, including the successful completion of preclinical studies and clinical trials and the time to completing an IPO or sale. Significant changes to the key assumptions used in the valuations could result in different fair values of common stock at each valuation date.

Segment and geographic information

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker, or decision-making group, in deciding how to allocate resources and in assessing performance. The Company views its operations and manages its business in one operating segment, which is the business of developing drugs that target cancer stem cells, and the Company operates in only one geographic area.

Comprehensive loss

Comprehensive loss is defined as the change in equity of a business enterprise during a period from transactions and other events and circumstances from non-owner sources. Comprehensive loss includes net loss and unrealized losses on investments.

Cash and cash equivalents

The Company considers all highly liquid investments with an original or remaining maturity of three months or less at the date of purchase to be cash equivalents. Cash equivalents consist of money market funds, U.S. agency notes and corporate bonds.

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

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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 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**

There were no financial instruments recorded at fair value as of December 31, 2010. The carrying amounts of accounts payable and accrued expenses approximate their fair values due to their short-term maturities.

In connection with the license with Poniard Pharmaceuticals Inc., (Poniard), as more fully described in Note 10, License Agreements, the Company is obligated to issue a warrant to Poniard 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. If the Company is publicly traded, 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.

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

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stock. The obligation to issue the warrant is revalued at the end of each reporting period, with changes in the fair value reported in research and development expense within the statement of operations.

As of November 17, 2011, the effective date of the obligation to issue the warrant, the most recent issuance of the Company s Preferred Stock had been the issuance of the Series C Preferred Stock in November 2011. As of December 31, 2011, there were no additional issuances of preferred stock.

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:

	November 17, 2011		December 31, 2011	
	Non-IPO	IPO	Non-IPO	IPO
Exercise price	$ 6.86	$ 10.00	$ 6.86	$ 10.00
Estimated stock price contingent upon milestone achievement	$ 3.19	$ 8.93	$ 3.22	$ 8.54
Expected term	4.2 years	4.2 years	4.1 years	4.1 years
Volatility	70%	70%	70%	70%
Dividend yield	0.00%	0.00%	0.00%	0.00%
Risk-free rate	0.64%	0.64%	0.60%	0.60%
Probability of achieving milestone	80%	80%	80%	80%
Probability of scenario	20%	80%	20%	80%

The fair value of the obligation to issue the warrant was recorded at $439,000. As a result of the change in inputs to the valuation model, the fair value of the obligation to issue the warrant decreased by $33,000 to $406,000 at December 31, 2011.

Investments

Investments and cash equivalents consist of investments in money market accounts, government-sponsored enterprise securities and commercial paper of publicly traded companies secured by the U.S. government that 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 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, which is a separate component of stockholders deficit, until such gains and losses are realized. The fair value of these securities is based on quoted prices for identical or similar assets. If a decline in the fair value is considered other-than-temporary, based on

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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 2011 or 2010. Realized gains and losses are determined using the specific identification method and are included in interest income in the statement of operations. There were no realized gains or losses recognized during 2011 or 2010.

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 December 31, 2011, 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.

As of December 31, 2011, cash, cash equivalents and investments included (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
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

The Company did not hold any cash equivalents or investments as of December 31, 2010.

Concentrations of credit risk and off-balance sheet risk

Cash and cash equivalents, short-term investments and long-term investments are financial instruments that potentially subject the Company to concentrations of credit risk. As of December 31, 2011, the Company s cash, cash equivalents and investments were deposited at two financial institutions. As of December 31, 2010, substantially all of the Company s cash was deposited in accounts at a single financial

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institution. The Company maintains its cash and cash equivalents and investments with high quality, accredited financial institutions and, accordingly, the Company believes it is not exposed to any significant credit risk on these funds. The Company has no significant off-balance sheet concentrations of credit risk, such as foreign currency exchange contracts, option contracts or other hedging arrangements.

Property and equipment

Property and equipment consists of laboratory equipment, office furniture, and computer equipment. Expenditures for repairs and maintenance are recorded to expense as incurred, whereas major betterments are capitalized as additions to property and equipment. Depreciation is calculated using the straight-line method over the following estimated useful lives of the assets:

Laboratory equipment	5 years
Furniture	5 years
Computer equipment	3 years

Upon retirement or sale, the cost of the disposed asset and the related accumulated depreciation are removed from the accounts and any resulting gain or loss is recognized.

The Company reviews its long-lived assets for impairment whenever events or changes in business circumstances indicate that the carrying value of assets may not be fully recoverable and that the useful lives of these assets are no longer appropriate. Each impairment test is based on a comparison of the undiscounted cash flow to the recorded value of the asset. If impairment is indicated, the asset will be written down to its estimated fair value. To date, no such impairment losses have been recorded.

Redeemable convertible preferred stock

The carrying value of the Company s Series A, Series B and Series C redeemable convertible preferred stock is adjusted by periodic accretions of offering costs such that the carrying value will equal the redemption amount at the earliest redemption date. The carrying value is also adjusted to reflect dividends when and if declared by the board of directors. No dividends have been declared by the board of directors since inception.

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.

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Organizational costs

All organizational costs have been expensed as incurred.

Research and development costs

The Company expenses research and development costs to operations as incurred. Research and development expenses consist of costs associated with research activities, including drug discovery efforts and the development of therapeutic product candidates and companion diagnostics. The Company accounts for nonrefundable advance payments for goods and services that will be used in future research and development activities as expenses when the service has been performed or when the goods have been received rather than when the payment is made. Research and development expenses consist of:

- employee-related expenses, including salaries, benefits, travel and stock-based compensation expense;
- external research and development expenses incurred under arrangements with third parties, such as contract research organizations, or CROs, manufacturing organizations and consultants, including the scientific advisory board;
- license fees; and
- facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, depreciation of leasehold improvements and equipment, and laboratory and other supplies.

Stock-based compensation

The Company expenses the fair value of employee stock options over the requisite service period, which is the vesting period. Compensation expense is measured using the fair value of the award at the grant date, net of estimated forfeitures, and is adjusted annually to reflect actual forfeitures. The grant date fair value of each stock-based award is estimated using the Black-Scholes option valuation model and is expensed on a straight-line basis over the vesting period.

Stock-based awards issued to nonemployees, including directors for non-board related services, are accounted for based on the fair value of such services received or of the equity instruments issued, whichever is more reliably measured. These stock-based option awards are revalued at

each vesting date using the Black-Scholes option valuation model and are expensed on a straight-line basis over the vesting period.

Prior to becoming a public company, the exercise prices for options granted were set by the board of directors, the members of which have extensive experience in the life science industry, with input from management of the Company, based on the board s determination of fair value of the common stock at the time of the grants. The Company performed contemporaneous valuations, utilizing a combination of valuation methods described in the American Institute of Certified Public Accountants Technical Practice Aid, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation,* (Practice Aid).

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Income taxes

The Company accounts for income taxes under the asset and liability method. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases using enacted tax rates in effect for the year in which the differences are expected to affect taxable income. Tax benefits are recognized when it is more likely than not that a tax position will be sustained during an audit. Deferred tax assets are reduced by a valuation allowance if current evidence indicates that it is considered more likely than not that these benefits will not be realized.

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 share and per share data):

	Year ended December 31, 2011	Period from August 4, 2010 (inception) through December 31, 2010	Period from August 4, 2010 (inception) to December 31, 2011
Net loss	$ (13,683)	$ (784)	$ (14,467)
Accretion of redeemable convertible preferred stock	(32)	(2)	(34)
Net loss applicable to common stockholders	$ (13,715)	$ (786)	$ (14,501)
Weighted-average number of common shares used in net loss per share applicable to common stockholders basic and diluted	1,295	850	1,171
Net loss per share applicable to common stockholders basic and diluted	$ (10.59)	$ (0.91)	$ (12.39)

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

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	Year ended December 31, 2011	Period from August 4, 2010 (inception) to December 31, 2010	Period from August 4, 2010 (inception) to December 31, 2011
Preferred stock	11,741	1,143	11,741
Outstanding stock options	405	177	405
Unvested restricted stock	1,435	2,009	1,435

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. The provisions of this ASU are effective prospectively for interim and annual periods beginning on or after December 15, 2011. Early application is prohibited. The Company does not expect the adoption of these provisions to have a significant impact on the financial statements.

In June 2011, the FASB issued ASU No. 2011-05, Comprehensive Income. This ASU intends to enhance 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 deficit. The provisions of this ASU will be applied retrospectively for interim and annual periods beginning after December 15, 2011. Early application is permitted. The Company does not expect the adoption of this standard to have a significant impact on the financial statements, but it will impact the manner in which the Company reports comprehensive loss.

3. Property and equipment

Property and equipment and related accumulated depreciation are as follows (in thousands):

	December 31, 2011	December 31, 2010
Laboratory equipment	$ 721	$ 8
Computer equipment	27	
Furniture	44	
	792	8
Less: accumulated depreciation	(83)	
	$ 709	$ 8

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Depreciation expense was $83,000 for the year ended December 31, 2011 and for the period from August 4, 2010 (inception) to December 31, 2011. The Company did not record any depreciation expense in the period from August 4, 2010 (inception) to December 31, 2010.

4. 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.

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) are being accreted through the earliest redemption date.

As described more fully in Note 13, Subsequent Events, the Company completed its initial public offering in February 2012 and all shares of its Preferred Stock converted into 11,740,794 shares of common stock.

The Company assessed the Preferred Stock for any beneficial conversion features or embedded derivatives that would require bifurcation from the Preferred Stock and receive separate accounting treatment. On the date of each issuance, the value of the common stock into which the Preferred Stock is convertible had a fair value less than the effective conversion price of the Preferred Stock and, as such, there was no intrinsic value on the respective commitment dates. No embedded derivatives were identified that would require bifurcation.

The rights, preferences, and privileges of Preferred Stock are as follows:

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Conversion

Shares of Preferred Stock are convertible into common stock based on a defined conversion ratio, which is originally set at one-for-one, adjustable for certain dilutive events. Conversion is at the option of the holders of Preferred Stock (Preferred Stockholders) at anytime without any additional considerations, although conversion is automatic upon the earlier of the sale of shares of common stock to the public at a price of at least $10.50 per share, for gross proceeds of at least $35 million, and where the shares are traded on either the New York Stock Exchange or NASDAQ or upon the written consent of holders of at least 60% of the outstanding Preferred Stock. During 2012, the Company s stockholders approved a reduction in the per share price required for the automatic conversion of the Preferred Stock into common stock to the public from $10.50 per share to $8.75 per share.

Dividends

Prior to the payment of any dividend, except a common stock dividend, to the common stockholders, the Preferred Stockholders are entitled to receive an amount at least equal to the amount that would have been received by the Preferred Stockholders had all shares of Preferred Stock been converted to common stock immediately prior to issuance of the dividend.

Liquidation preference

In the event of any liquidation, dissolution or winding up of the Company, including a deemed liquidation event, such as certain mergers or a disposition of substantially all the assets of the Company, unless holders of at least 60% of the outstanding Preferred Stock elect otherwise, the Preferred Stockholders are entitled to receive, in preference to common stockholders, an amount equal to the Original Issue Price ($1.00 per share for Series A Preferred Stock, $2.00 per share for Series B Preferred Stock and $2.25 per share for Series C Preferred Stock, adjustable for certain dilutive events) plus all declared but unpaid dividends. If the Company has insufficient assets to pay the Preferred Stockholders the full amount to which they are entitled, the Preferred Stockholders share ratably in any distribution in proportion to the respective amounts which would otherwise be payable.

After payment of these preferential amounts, the remaining assets of the Company are distributable ratably to the holders of common stock and Preferred Stock on an as-converted to common basis. However, the Preferred Stockholders are limited to the receipt of an aggregate amount (including through payment of the preferential amounts described above) equal to the greater of:

(1) 1.75 times the aggregate amount of the applicable Original Purchase Price, and

(2) the amount the Preferred Stockholder would have received if all Preferred Stock had been converted to common stock immediately prior to the liquidation event.

Voting rights

Holders of the Preferred Stock are entitled to vote as a single class with the holders of common stock, and have one vote for each equivalent common share into which the Preferred Stock is convertible. A 60% vote of the Preferred Stockholders in addition to a 60% vote of Series A

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Preferred Stock and Series B Preferred Stock voting as a single class, is required in order to effect a liquidation, reclassification or recapitalization of the Company s capital stock or a deemed liquidation event, such as certain mergers or a disposition of substantially all the assets of the Company, amend the certificate of incorporation or bylaws, create or issue shares of another class of stock that is pari passu or senior to the Preferred Stock, repurchase or redeem or pay any dividend on any capital stock, subject to limited exceptions, issue any debt security such that the Company s aggregate indebtedness would exceed $1 million, acquire capital stock of another entity, increase or decrease the authorized number of directors or increase the number of shares of common stock reserved under the Company s equity incentive plan. The holders of the Series A Preferred Stock are entitled to elect four directors, the Preferred Stockholders and common stockholders, voting as one class on an as-converted basis, are entitled to elect two directors, and the common stockholders are entitled to elect one director.

Redemption

The Preferred Stock is redeemable at the applicable Original Issue Price plus any declared but unpaid dividends. The Series C Preferred Stock is redeemable beginning in 2016 at the demand of specific holders of the Series C Preferred Stock. The Series B Preferred Stock is redeemable beginning in 2016 at the demand of holders of at least two-thirds of the Series B Preferred Stock. The Series A Preferred Stock is redeemable upon the redemption of another series of Preferred Stock at the demand of holders of at least two-thirds of the Series A Preferred Stock. The redemption for the Preferred Stock is payable in three equal annual installments.

5. Common stock

As of December 31, 2011 and December 31, 2010, the Company had reserved the following shares of common stock for the potential conversion of outstanding Preferred Stock and the exercise of stock options (in thousands):

	December 31, 2011	December 31, 2010
Series A Preferred Stock	4,571	4,571
Series B Preferred Stock	4,579	
Series C Preferred Stock	2,591	
Shares reserved under equity compensation plans	563	405
	12,304	4,976

Each share of common stock is entitled to one vote, subject to certain voting rights of the Preferred Stock as discussed in Note 4. The holders of the common stock are also entitled to receive dividends whenever funds are legally available and when declared by the board of directors, subject to the prior rights of the Preferred Stockholders.

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Common stock issued for license

The Company issued 166,664 shares of common stock in the period from August 4, 2010 (inception) to December 31, 2010 and in the period from August 4, 2010 (inception) to December 31, 2011 in exchange for certain intellectual property rights. The fair value of the common stock was determined to be $0.28 per share and the fair value was determined to be more readily determinable than the fair value of the license. As a result, the fair value of the shares of approximately $46,000 was recorded as research and development expense.

6. Stock-based compensation

In November 2010, the Company adopted the Verastem, Inc. 2010 Equity Incentive Plan (the 2010 Plan) under which it may grant incentive stock options (ISOs), nonstatutory stock options (NSOs), restricted stock awards, restricted stock unit awards and stock appreciation rights to purchase up to 404,762 shares of common stock to eligible employees, officers, directors and consultants. In March 2011, the Company increased the number of shares of common stock available under the 2010 Plan to 571,242 shares. As of December 31, 2011, 30,101 shares are available for future issuance under the 2010 Plan. Terms of stock option agreements, including vesting requirements, are determined by the board of directors, subject to the provisions of the 2010 Plan. Generally, options granted by the Company vest over four years, expire no later than ten years from the date of grant and have an exercise price equal to the estimated fair value of the common stock as determined by the board of directors on the date of grant.

In December 2011, the Company adopted the 2012 Incentive Plan (the 2012 Plan). The 2012 Plan became effective immediately upon the closing of the Company s IPO in February 2012. The 2012 Plan provides for the grant of ISOs, NSOs, 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 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 common stock

In August 2010, the Company issued 2.9 million shares of its common stock to the founders at a purchase price of $0.00035 per share, determined to be the fair value of the common stock on the date of issuance. The shares were issued under restricted stock purchase agreements, which allow the Company, at its discretion, to repurchase unvested shares if the founders terminate their relationship with the Company. Upon execution of the restricted stock purchase agreements, 25% of the shares vested immediately and the remaining shares vest ratably on a quarterly basis over a four year term.

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During the year ended December 31, 2011, the Company issued 256,000 shares of its common stock to new employees of the Company at a purchase price of $0.28 per share, determined to be the fair value of the common stock on the date of issuance. The shares were issued under the terms of the Plan, and allow the Company, at its discretion, to repurchase unvested shares if the employees terminate their relationship with the Company. The shares vest over a four year term, with 25% vesting after the first year and the remainder vesting ratably on a monthly basis for the remaining three years. The purchase price received for the shares was not material to the financial statements. The shares are recorded in stockholders deficit as they vest.

The Company records stock-based compensation expense for the common stock subject to repurchase based on the grant date intrinsic value for employees and the vesting date intrinsic value for non-employees. All of the restricted shares were issued at fair value. The Company has recorded stock-based compensation expense of $1.4 million, $51,000 and $1.5 million for the year ended December 31, 2011, for the period from August 4, 2010 (inception) to December 31, 2010 and for the period from August 4, 2011 (inception) to December 31, 2011, respectively, associated with restricted common stock. The $1.4 million recorded for the year ended December 31, 2011 includes $34,000 associated with modifications to certain restricted stock purchase agreements.

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

	Shares	Weighted-average purchase price per share
Outstanding at August 4, 2010		$
Granted	2,857	0.00035
Vested	(848)	0.00035
Outstanding at December 31, 2010	2,009	0.00035
Granted	256	0.2800
Vested	(544)	0.0045
Forfeited	(286)	0.1173
Outstanding at December 31, 2011	1,435	0.0253

Stock options

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

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	Shares	Weighted-average price per share	Weighted-average remaining contractual term (years)	Aggregate intrinsic value
Outstanding at August 4, 2010		$		
Granted	177	0.28		
Outstanding at December 31, 2010	177	0.28	9.9	$ 12
Granted	228	1.12		
Outstanding at December 31, 2011	405	0.75	9.3	$ 176
Exercisable at December 31, 2010		$ 0.28	9.9	$ 12
Exercisable at December 31, 2011	46	$ 0.28	8.9	$
Vested and expected to vest at December 31, 2010	177	$ 0.28	9.9	$ 12
Vested and expected to vest at December 31, 2011	405	$ 0.75	9.3	$ 176

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:

	Year ended December 31, 2011	2010
Risk-free interest rate	1.1-2.7%	2.0%
Dividend yield		
Volatility	69-70%	67%
Expected term (years)	6.0-6.1	6.1

The Company uses the simplified method as prescribed by the Securities and Exchange Commission Staff Accounting Bulletin No. 107, *Share-Based Payment*, to calculate the expected term as it does not have sufficient historical exercise data to provide a reasonable basis upon which to estimate the expected term for options granted to employees and utilizes the contractual term for options granted to non-employees. The expected term is applied to the stock option grant group as a whole, as the Company does not expect substantially different exercise or post-vesting termination behavior among its employee population. The computation of expected volatility is based on the historical volatility of a representative group of companies with similar characteristics to us, including early stage of product development and therapeutic focus. The representative group of companies consisted of Alnylam Pharmaceuticals, Inc., Anadys Pharmaceuticals, Inc., ARIAD Pharmaceuticals, Inc., Curis Inc., Cytokinetics, Inc., Exelixis, Inc., Geron, Corp., Infinity Pharmaceuticals, Inc., Momenta Pharmaceuticals, Inc. and Oncothyreon, Inc. The risk-free interest rate is based on a treasury instrument whose term is consistent with the expected life of the stock options. Management estimates expected forfeitures based on data from a representative group of companies with similar characteristics to us and recognizes compensation costs only for those equity awards expected to vest.

For the period from August 4, 2010 (inception) to December 31, 2010, the Company did not recognize any stock-based compensation for employee stock option grants. The Company recognized total stock-based compensation expense for employee stock option grants of $19,000

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in the year ended December 31, 2011 and the period from August 4, 2010 (inception) to December 31, 2011. The weighted-average grant date fair value of options granted in the period from August 4, 2010 (inception) to December 31, 2010, the year ended December 31, 2011 and the period from August 4, 2010 (inception) to December 31, 2011 was $0.18, $0.75 and $0.60 per share, respectively.

Stock-based awards issued to nonemployees, including directors for non-board related services, are accounted for using the fair value method. These stock-based option awards are revalued on each vesting and reporting date. The Company recognized total stock-based compensation expense of approximately $209,000, $1,000, and $210,000 in the year ended December 31, 2011, the period from August 4, 2010 (inception) to December 31, 2010, and the period from August 4, 2010 (inception) to December 31, 2011, respectively. Due to an operating loss, the Company does not record tax benefits associated with stock-based compensation and option exercises. Tax benefits will be recorded when realized.

At December 31, 2011, there was $810,000 of total unrecognized compensation cost related to nonvested stock options, respectively. As of December 31, 2011, the Company expects to recognize these costs over a remaining weighted-average period of 2.9 years.

7. Income taxes

As of December 31, 2011 the Company had federal net operating loss carryforwards of approximately $11.6 million and state net operating loss carryforwards of $11.9 million, which are available to reduce future taxable income. The Company also had federal tax credits of $358,000 and state tax credits of $42,000, which may be used to offset future tax liabilities. The net operating loss (NOL) and tax credit carryforwards will expire at various dates through 2031. Net operating loss and tax credit carryforwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities and may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant shareholders over a three-year period in excess of 50%, as defined under Sections 382 and 383 of the Internal Revenue Code, as well as similar state provisions. This could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on the value of the Company immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation in future years.

A reconciliation of income taxes computed using the U.S. federal statutory rate to that reflected in operations follows:

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	Year ended December 31,	
	2011	2010
Income tax benefit using U.S. federal statutory rate	34.00%	34.00%
State income taxes, net of federal benefit	4.78%	5.62%
Research and development tax credits	1.65%	1.96%
Permanent items	(4.78)%	(0.78)%
Change in the valuation allowance	(35.65)%	(40.80)%
Other	%	%
	%	%

The principal components of the Company s deferred tax assets are as follows (in thousands):

	December 31,	
	2011	2010
Deferred tax assets:		
Net operating loss carryforwards	$ 4,562	$ 225
Capitalized research and development	148	55
Research and development credits	385	18
Stock-based compensation	82	20
Other	20	2
Gross deferred tax assets	5,197	320
Valuation allowance	(5,197)	(320)
Net deferred tax asset	$	$

The Company has recorded a valuation allowance against its deferred tax assets at December 31, 2011 because the Company s management believes that it is more likely than not that these assets will not be fully realized. The increase in the valuation allowance of $4.9 million in 2011 primarily relates to the net loss incurred by the Company.

Upon inception, the Company adopted accounting guidance related to accounting for uncertainty in income taxes. The Company s reserves related to taxes are based on a determination of whether and how much of a tax benefit taken by the Company in its tax filings or positions is more likely than not to be realized following resolution of any potential contingencies present related to the tax benefit. Upon adoption, the Company recognized no material adjustment for unrecognized income tax benefits. As of the adoption date and through December 31, 2011, the Company had no unrecognized tax benefits or related interest and penalties accrued. The Company has not, as yet, conducted a study of research and development (R&D) credit carryforwards. This study may result in an adjustment to the Company s R&D credit carryforwards; however, until a study is completed and any adjustment is known, no amounts are being presented as an uncertain tax position. A full valuation allowance has been provided against the Company s R&D credits and, if an adjustment is required, this adjustment would be offset by an adjustment to the valuation allowance. Thus, there would be no impact to the balance sheet or statement of operations if an adjustment were required. The Company would recognize both accrued interest and penalties related to unrecognized benefits in income tax expense. The Company s uncertain tax positions are related to years that remain subject to examination by relevant tax authorities. Since the Company is in a loss carryforward position, the Company is generally subject to examination by the U.S. federal, state and local income tax authorities for all tax years in which a loss carryforward is available.

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8. Commitments and contingencies

From November 2010 through May 2011, the Company leased office space from a shareholder. There was no formal lease arrangement with the shareholder. Rent paid to the shareholder was $34,000, $12,000, and $46,000 for the year ended December 31, 2011, the period from August 4, 2010 (inception) to December 31, 2010, and the period from August 4, 2010 (inception) to December 31, 2011, respectively.

In May 2011, the Company entered into a non-cancelable operating lease for office and laboratory space, which expires October 31, 2014. The lease agreement provides for free rent for the first four months of the lease term and includes escalating rent payments. The rent expense is recorded on a straight-line basis over the lease term. The Company is also obligated to pay for certain operating costs and a proportional share of certain common area costs. The Company has the right to extend the lease for a two-year period. The annual rent for each additional year is determined annually at the then fair market rate. The Company secured a letter of credit for $86,000 in connection with the lease, which is included in restricted cash on the balance sheet. The minimum aggregate future lease commitments are as follows (in thousands):

2012	$	351
2013		360
2014		307
	$	1,018

The Company recorded rent expense of $251,000, $12,000 and $263,000 for the year ended December 31, 2011, the period from August 4, 2010 (inception) to December 31, 2010, and the period from August 4, 2010 (inception) to December 31, 2011, respectively.

9. Accrued expenses

Accrued expenses consist of the following (in thousands):

	December 31,	
	2011	**2010**
Professional fees	$ 520	$ 35
Contract research organizations	217	
Compensation and related benefits	86	15
Deferred rent	27	
License fees		30
Other expenses	23	9
	$ 873	$ 89

10. License agreements

In October 2010, the Company entered into an exclusive license agreement, which was amended and restated in January 2012, with the Whitehead Institute for Biomedical Research (the

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Licensor) for certain intellectual property. The Company paid the Licensor an upfront license fee and reimbursed patent related fees and costs incurred by the Licensor and affiliates of the Licensor totaling $104,000 in the aggregate and issued 166,664 shares of common stock to the Licensor and entities and individuals affiliated with the Licensor. The fair value of the common stock was determined to be $0.28 per share, and the fair value was determined to be more readily determinable than the fair value of the license. As a result, the fair value of the shares of approximately $46,000 was recorded as research and development expense. Under the terms of the agreement, the Company also agreed to pay annual license maintenance fees, milestone payments, royalties as a percentage of net sales and a percentage of sublicense income the Company receives. Annual license maintenance fees are creditable against royalties earned during the same calendar year and are not material to the financial statements. Milestone payments are triggered upon the achievement of specified development, regulatory and commercialization milestones and are not creditable against royalties. Actual amounts due under the agreement will vary depending on the number of products developed, the type and development path of the products, and other related factors. Milestone payments could total up to $1.6 million. The Company may terminate the agreement at any time with 90 days prior written notice.

On November 17, 2011, the Company entered into an exclusive, worldwide license agreement with Poniard Pharmaceuticals, Inc. to develop, make, use and sell compounds and products covered by the licensed patent rights for the diagnosis, treatment, prevention or control of human diseases and conditions. Under the agreement, the Company paid an upfront license fee and agreed to pay $13.3 million upon the achievement of specified development and regulatory milestones. The Company also agreed to issue to Poniard a warrant to purchase 142,857 shares of common stock upon the first dosing of the first patient in a Phase 1 clinical trial of a licensed product. The exercise price of such warrant would be equal to the average closing price of the Company s common stock during the five trading days preceding such issue date. In addition, the Company agreed to pay royalties as a percentage of net sales of licensed products. The Company may terminate the agreement at any time with 90 days prior written notice.

On December 16, 2011, the Company amended and restated an existing non-exclusive license agreement with the Licensor pursuant to which the Company obtained an exclusive license to certain intellectual property. The Company paid the Licensor an upfront license fee and agreed to make milestone payments of up to $825,000 upon the achievement of specified regulatory and commercialization milestones. In addition, the Company agreed to pay royalties as a percentage of net sales of licensed products.

11. Employee benefit plan

In June 2011, the Company adopted a 401(k) retirement and savings plan (the 401(k) Plan) covering all employees. The 401(k) Plan allows employees to make pre-tax contributions up to the maximum allowable amount set by the IRS. Under the 401(k) Plan, the Company may make discretionary contributions as approved by the board of directors. During the year ended December 31, 2011 and the period from August 4, 2010 (inception) to December 31, 2011, the Company made contributions to the 401(k) Plan of $46,000.

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12. Quarterly Financial Information (unaudited, in thousands, except per share data)

	First Quarter Ended March 31, 2011	Second Quarter Ended June 30, 2011	Third Quarter Ended September 30, 2011	Fourth Quarter Ended December 31, 2011
Operating expenses:				
Research and development	$ 675	$ 1,726	$ 3,082	$ 4,400
General and administrative	471	759	965	1,620
Total operating expenses	1,146	2,485	4,047	6,020
Loss from operations	(1,146)	(2,485)	(4,047)	(6,020)
Interest income				15
Net loss	$ (1,146)	$ (2,485)	$ (4,047)	$ (6,005)
Net loss per share applicable to common stockholders-basic and diluted	$ (1.06)	$ (2.03)	$ (2.98)	$ (4.01)
Weighted average number of common shares used in net loss per share applicable to common stockholders-basic and diluted	1,089	1,225	1,361	1,500

	Period from August 4, 2010 (inception) to September 30, 2010	Fourth Quarter Ended December 31, 2010
Operating expenses:		
Research and development	$	$ 400
General and administrative	84	300
Total operating expenses	84	700
Loss from operations	(84)	(700)
Interest income		
Net loss	$ (84)	$ (700)
Net loss per share applicable to common stockholders-basic and diluted	$ (0.12)	$ (0.77)
Weighted average number of common shares used in net loss per share applicable to common stockholders-basic and diluted	714	924

The Company was incorporated on August 4, 2010; however, it did not commence operations until the fourth quarter of 2010. Activity incurred during the period from August 4, 2010 (inception) to September 30, 2010 is limited to organizational costs which were expensed when incurred.

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13. Subsequent events

The Company reviews all activity subsequent to year end but prior to the issuance of the financial statements for events that could require disclosure or that could impact the carrying value of assets or liabilities as of the balance sheet date. All significant subsequent events have been properly disclosed in the financial statements.

In January 2012, the Company s stockholders approved a reduction in the per share price required for the automatic conversion of the Preferred Stock into common stock upon the sale of shares of common stock to the public from $10.50 per share to $8.75 per share.

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 price of $10.00 per share, including the over-allotment option. Net proceeds of the IPO were $56.7 million. In conjunction with this transaction, all shares of the Company s Preferred Stock were converted into 11,740,794 shares of common stock.

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EXHIBIT INDEX

Exhibit number	Description of exhibit
3.1*	Amended and Restated Certificate of Incorporation, as amended, of the Registrant
3.2	Bylaws of the Registrant (incorporated by reference to Exhibit 3.4 to Amendment No. 3 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on January 13, 2012)
4.2	Second Amended and Restated Investors Rights Agreement, dated November 1, 2011, by and among the Registrant and the other parties thereto (incorporated by reference to Exhibit 4.2 to Amendment No. 3 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on January 13, 2012)
10.1#	2010 Equity Incentive Plan (incorporated by reference to Exhibit 10.1 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on November 3, 2011)
10.2#	2012 Incentive Plan (incorporated by reference to Exhibit 10.2 to Amendment No. 3 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on January 13, 2012)
10.3#	Form of Incentive Stock Option Agreement under 2012 Incentive Plan (incorporated by reference to Exhibit 10.3 to Amendment No. 3 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on January 13, 2012)
10.4#	Form of Nonqualified Stock Option Agreement under 2012 Incentive Plan (incorporated by reference to Exhibit 10.4 to Amendment No. 3 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on January 13, 2012)
10.5#	Form of Restricted Stock Unit Agreement under 2012 Incentive Plan (incorporated by reference to Exhibit 10.16 to Amendment No. 3 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on January 13, 2012)
10.6#	Amended and Restated Employment Agreement between the Registrant and Robert Forrester (incorporated by reference to Exhibit 10.5 to Amendment No. 3 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on January 13, 2012)
10.7#	Amended and Restated Employment Agreement between the Registrant and Jonathan Pachter (incorporated by reference to Exhibit 10.6 to Amendment No. 3 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on January 13, 2012)
10.8#	Form of Indemnification Agreement between the Registrant and each director (incorporated by reference to Exhibit 10.7 to Amendment No. 1 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on December 7, 2011)
10.9	Lease Agreement, dated May 2, 2011, between the Registrant and ARE-MA Region No. 38, LLC (incorporated by reference to Exhibit 10.8 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on November 3, 2011)
10.10	Amended and Restated Exclusive Patent License Agreement and Tangible Property Agreement, dated January 11, 2012, by and among the Registrant and the Whitehead

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	Institute for Biomedical Research (incorporated by reference to Exhibit 10.9 to Amendment No. 3 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on January 13, 2012)
10.11	Exclusive Patent License Agreement, dated December 16, 2011, by and among the Registrant and the Whitehead Institute for Biomedical Research (incorporated by reference to Exhibit 10.10 to Amendment No. 2 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on December 22, 2011)
10.12	License Agreement, dated November 17, 2011, between the Registrant and Poniard Pharmaceuticals, Inc. (incorporated by reference to Exhibit 10.15 to Amendment No. 2 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on December 22, 2011)
10.13	Letter Agreement, dated October 1, 2010, between the Registrant and the Broad Institute (incorporated by reference to Exhibit 10.11 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on November 3, 2011)
10.14#	Letter Agreement, dated July 30, 2010, as amended October 18, 2010, between the Registrant and Piyush Gupta, Ph.D. (incorporated by reference to Exhibit 10.12 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on November 3, 2011)
10.15#	Letter Agreement, dated August 20, 2010, between the Registrant and Eric Lander, Ph.D. (incorporated by reference to Exhibit 10.13 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on November 3, 2011)
10.16#	Letter Agreement, dated July 30, 2010, as amended October 18, 2010, between the Registrant and Robert Weinberg, Ph.D. (incorporated by reference to Exhibit 10.14 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on November 3, 2011)
10.17*#	Restricted Stock Purchase Agreement, dated August 11, 2010, between the Registrant and Christoph Westphal
10.18*#	Restricted Stock Purchase Agreement, dated August 11, 2010, between the Registrant and Richard Aldrich
10.19*#	Employment Agreement, dated March 28, 2012, between the Registrant and Paul Brannelly
31.1*	Certification of President and Chief Executive Officer pursuant to Exchange Act Rule 13a-14(a)
31.2*	Certification of Chief Financial Officer pursuant to Exchange Act Rule 13a-14(a)
32.1*	Certification of President and 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.

* Filed herewith.

Confidential treatment granted as to portions of the exhibit. Confidential materials omitted and filed separately with the Securities and Exchange Commission.

Management contract or compensatory plan, contract or agreement.