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Verastem, Inc. CONSOLIDATED FINANCIAL STATEMENTS

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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 10-K

(Mark One)

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

For the fiscal year ended December 31, 2014

or

☐ **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.)
117 Kendrick Street, Suite 500 Needham, Massachusetts (Address of principal executive offices)	**02494** (Zip Code)

Registrant's telephone number, including area code: **(781) 292-4200**

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. ☐ Yes ☒ 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. ☐ Yes ☒ 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. ☒ Yes ☐ No

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). ☒ Yes ☐ 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. ☐

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 ☐	Accelerated filer ☒	Non-accelerated filer ☐ (Do not check if a smaller reporting company)	Smaller reporting company ☐

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

Aggregate market value of the voting and non-voting common equity held by non-affiliates of the registrant as of June 30, 2014 was $218,641,564.

The number of shares outstanding of the registrant's common stock as of February 27, 2015 was 35,955,110.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of our definitive proxy statement to be delivered to stockholders in connection with the 2015 Annual Meeting of Stockholders are incorporated by reference into Part III of this Annual Report on Form 10-K.

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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 related to present facts or current conditions or 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. Such statements relate to, among other things, the development of our product candidates, including VS-6063, VS-4718 and VS-5584, and our FAK, PI3K/mTOR, and diagnostics programs generally, the timeline for clinical development and regulatory approval of our product candidates, the expected timing for the reporting of data from on-going trials and for the COMMAND (as defined in Item 1) interim analysis, the expected timing of completion of COMMAND enrollment, the structure of our planned or pending clinical trials, additional planned studies, our rights to develop or commercialize our product candidates and our ability to finance contemplated development activities and fund operations for a specified period. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue" and similar expressions are intended to identify forward-looking statements, although not all forward- looking statements contain these identifying words.

Forward-looking statements are not guarantees of future performance and our actual results could differ materially from the results discussed in the forward-looking statements. Factors that could cause actual results to differ materially from those in the forward-looking statements include, but are not limited to, our ability to raise additional capital to support our clinical development programs and other operations, our ability to develop products of commercial value and to identify, discover and obtain rights to additional potential product candidates, our ability to protect and maintain our intellectual property and the ability of our licensors to obtain and maintain patent protection for the technology or products that we license from them, the fact that the preclinical and clinical testing of our product candidates and preliminary data from clinical trials may not be predictive of the success of ongoing or later clinical trials, that data may not be available when we expect it to be, that enrollment of clinical trials may take longer than expected, that our product candidates may cause unexpected safety events, that we will be unable to successfully initiate or complete the clinical development of our product candidates, including VS-6063, VS-4718 and VS-5584, that development of our product candidates will take longer or cost more than planned, our reliance on third- parties, competitive developments, the effect of current and future legislation and regulation and regulatory actions, as well as other risks described under "Risk Factors" and elsewhere in this Annual Report on Form 10-K and other filings with the Securities and Exchange Commission, or SEC.

As a result of these and other factors, 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. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make. 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.

PART I

Item 1. Business

OVERVIEW

We are a biopharmaceutical company focused on discovering and developing drugs to treat cancer by the targeted killing of cancer stem cells. 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 tumors, their recurrence and metastasis. Our most advanced programs target the Focal Adhesion Kinase, or FAK, and the PI3K/mTOR signaling pathways. Our lead FAK inhibitor, VS-6063, has been assigned defactinib as the United States Adopted Name (USAN). We have received orphan drug designation for the use of VS-6063 in mesothelioma in the European Union and in the United States. VS-6063 is currently in a registration-directed trial (COMMAND) in patients with malignant pleural mesothelioma, a Phase 1b trial in combination with weekly paclitaxel for patients with ovarian cancer, a Phase 2 study in patients with non-small cell lung cancer, a Phase 2 trial preceding surgery in mesothelioma and a combination trial of VS-6063 and VS-5584 in patients with relapsed mesothelioma. We expect to conduct an interim analysis of COMMAND during the second quarter of 2015 and following this and assuming the trial is not futile, we intend to communicate the specific patient population for the primary analysis. We also expect to update or announce results from the combination trial of VS-6063 and paclitaxel in patients with ovarian cancer and results from the Phase 2 study of VS-6063 in patients with non-small cell lung cancer in the second half of 2015 and to report preliminary data on the extended treatment cohort for the Phase 2 trial preceding surgery in mesothelioma in the first half of 2016. In addition to VS-6063, both our FAK inhibitor VS-4718 and our dual mTORC1/2 and PI3K inhibitor VS-5584 are in Phase 1 clinical trials in patients with advanced cancers. We have received orphan drug designation for the use of VS-5584 in mesothelioma in the European Union and in the United States. We also expect to report results from the Phase 1 trials of VS-4718 and VS-5584 in the second half of 2015.

Cancer is a group of diseases characterized by uncontrolled growth and spread of abnormal cells. The American Cancer Society estimated that in the United States in 2014, approximately 1.7 million new cases of cancer would be diagnosed and nearly 600,000 people would 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 was projected to reach $124.6 billion. IMS Health estimates that in the United States in 2014, approximately $37.5 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 cancer therapies to achieve a durable clinical response is the presence of cancer stem cells, or CSCs, which are also sometimes referred to as tumor-initiating cells, within tumors. CSCs have been identified in many types of cancer, including mesothelioma, ovarian, 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 therapies failing. Accordingly, our mission is to develop drugs targeting CSCs that either in combination with other cancer treatments or alone can target all of the cells comprising a tumor and, thus, create a durable clinical response.

We have 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. Our most advanced product candidates are VS-6063, VS-4718 and VS-5584. We are currently evaluating these compounds

in both preclinical and clinical studies as potential therapies for certain cancers, including mesothelioma, ovarian and lung. We believe that these compounds may be especially beneficial as therapeutics in aggressive cancers driven by CSCs that have a poorer prognosis and lower overall survival rate than other types of cancer.

OUR MANAGEMENT TEAM AND SCIENTIFIC CO-FOUNDERS AND ADVISORS

Our experienced management team includes our Executive Chairman and co-founder Christoph Westphal, M.D., Ph.D., our President and Chief Executive Officer, Robert Forrester, our Chief Financial Officer, John "Jack" Green, C.P.A, our Chief Medical Officer, Joanna Horobin, M.B., Ch.B., our Chief Operating Officer, Daniel Paterson and our General Counsel, Monica Kleinman.

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., Momenta Pharmaceuticals, Inc., Acceleron Pharma Inc., and Flex Pharma, Inc. Dr. Westphal also co-founded Alnara Pharmaceuticals, Inc., which was acquired by Eli Lilly and Co. in 2010 and OvaScience, Inc.

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. and Coley Pharmaceutical Group, Inc., which was acquired by Pfizer Inc. in 2007.

Mr. Green was the Senior Vice President and Chief Financial Officer of GTC Biotherapeutics (formerly Genzyme Transgenics Corporation), which was spun out from Genzyme Corporation as a stand-alone public company. Mr. Green is a Certified Public Accountant (CPA) with over 30 years of financial experience, including 20 within the biotechnology industry.

Ms. Kleinman was a member of the securities and public company group at Ropes & Gray LLP where she advised on corporate transactions involving public companies, investment banks and private equity funds and provided counsel on securities law compliance and corporate governance matters. Ms. Kleinman holds a B.A. from Amherst College and a J.D. from Harvard Law School.

Dr. Horobin has over 30 years of pharmaceutical drug development experience in senior development and executive roles in both public and private companies. Dr. Horobin previously held positions as President and Chief Executive Officer at Syndax Pharmaceuticals, Vice President, Oncology at Rhone-Poulenc Rorer (now Sanofi,), Chief Operating Officer of CombinatoRx and Executive Vice President at EntreMed with experience spanning clinical development, marketing, and general management in both public and private companies. Dr. Horobin has been involved in the development, marketing and commercial launch of 10 approved products. Dr. Horobin received her medical degree from the University of Manchester.

Mr. Paterson has over 20 years of experience in management roles at healthcare and biotechnology companies, including as chief executive officer and chief operating officer, and specific expertise in oncology drug and diagnostic product development. Mr. Paterson was Head of Global Strategy for Specialty Market and Patient-Level Data at IMS Health after playing a key role in the acquisition of PharMetrics by IMS Health as VP, Marketing and Corporate Development.

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, Professor of Biology at MIT and Professor of Systems Biology at Harvard Medical School, played a central role in the Human Genome Project. Collaborative research in the labs of Dr. Lander and Dr. Weinberg developed our proprietary 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 and chair*	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
Jose Baselga, M.D., Ph.D. *Senior Medical Advisor*	Physician in Chief at Memorial Sloan Kettering Cancer Center. Formerly the Bruce A. Chabner Chair and Chief of the Division of Hematology/Oncology at Massachusetts General Hospital and associate director of the MGH Cancer Center
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
Richard Sackler, M.D.	Co-chairman, Purdue Pharma and Adjunct Professor of Genetics at Rockefeller University
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
Christopher Walsh, Ph.D.	Hamilton Kuhn Professor in the Department of Biological Chemistry and Molecular Pharmacology at Harvard Medical School
Max Wicha, M.D.	Director, University of Michigan Comprehensive Cancer Center and Distinguished Professor of Oncology at the University of Michigan
Eric Winer, M.D.	Director of the Breast Oncology Center at the Dana Farber Cancer Institute and Professor of Medicine at Harvard Medical School

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 estimated that in the United States in 2014, approximately 1.7 million new cases of cancer would be diagnosed and nearly 600,000 people would 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 was projected to reach $124.6 billion and the cancer type expected to be 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 2013 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	234,580	2,645,621
Lung and bronchus	228,190	373,489
Colorectal	142,820	1,110,077
Leukemia	48,610	253,350
Pancreatic	45,220	34,657
Brain and other nervous system cancers	23,130	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, 2004-2010, 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)	61%	98.6%
Regional (spread to regional lymph nodes)	32%	84.6%
Distant (cancer has metastasized)	5%	25.0%

(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.

Cancer stem cells

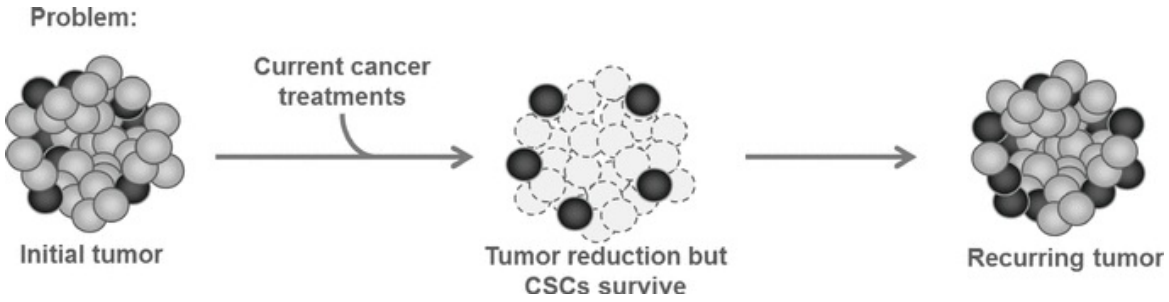
We believe that CSCs, which are sometimes referred to as tumor-initiating cells, are responsible for the initiation, metastasis and recurrence of many cancers. CSCs have the ability to:

- 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.



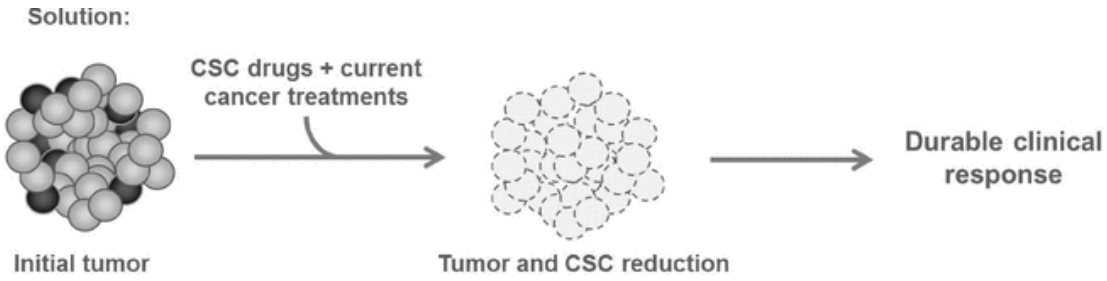
The need to target CSCs may apply across the treatment of a broad range of cancers. CSCs have been isolated and characterized from many types of cancer, including mesothelioma, ovarian, breast, pancreatic, colon, brain, lung and leukemia. The CSCs isolated from each of these tumor types have been found 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. FAK is a central signaling node in the development of CSCs and critical for the establishment of metastases. In 2012, research conducted in Dr. Weinberg's laboratory and published in Cancer Discovery elucidated the role of FAK in breast cancer disease progression. This work found that increased FAK activity leads to increases in the metastatic capability of cancer cells following the process of epithelial to mesenchymal transition, an underlying mechanism in CSC development. Similarly, the role of PI3K/mTOR signaling has also been linked to disease progression and the survival of CSCs in multiple types of solid tumors and leukemia.

An increase in the proportion of CSCs in response to chemotherapy has been observed in clinical studies. For example, 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 a combination of treatments 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 CSCs. We believe that by developing therapeutics that target CSCs we can address the problem of cancer recurrence and metastasis. To achieve a durable clinical response, we believe that it may be necessary to target 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.



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 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 initiating new tumors. We and scientists at the Whitehead Institute and the Broad Institute have used our technology and high-throughput screening methods to evaluate the ability of over 300,000 compounds to kill CSCs.

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 targeting CSCs. Key elements of our strategy to achieve this goal are:

- Execute on the registration-directed trial of VS-6063 in mesothelioma named COMMAND (Control of Mesothelioma with MAinteNance Defactinib). We have met with the regulatory agencies in the United States, the United Kingdom and Japan and interacted with regulatory agencies in other countries worldwide. Based on these discussions, we believe that positive results from this trial, if obtained, may enable us to seek regulatory approval for VS-6063 in mesothelioma.

- Rapidly advance our product candidates through clinical development. We have initiated clinical trials of VS-6063 both as a single agent and in combination with other agents and are conducting additional clinical trials with VS-4718 and VS-5584. We expect to initiate further clinical trials over the next several years.

- Expand the indications in which our product candidates may be used. In parallel to the mesothelioma, ovarian and non-small cell lung cancer trials that we are currently conducting, we plan to pursue additional disease indications as we believe CSCs are implicated in many types cancers, including breast, pancreatic, colon, brain, lung and leukemia.

- Collaborate selectively to augment and accelerate translational research, development and commercialization. We may seek third-party collaborators for the development and eventual 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.

- Consider acquiring or in-licensing rights to additional compounds. We may pursue the acquisition or in-license from third parties of rights to additional compounds. We believe that our approach of identifying CSC targeting product 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 candidates.

- Maintain scientific leadership in the CSC field. We plan to continue to conduct research in the CSC field 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 exceptional advisors, employees and management are critical to leadership in the CSC field.

OUR PRODUCT CANDIDATES

Overview

We are focused on the discovery and development of small molecules to allow flexibility in the design of molecules for optimized efficacy and safety primarily as orally available drug 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 have three product candidates currently in clinical trials including the focal adhesion kinase (FAK) inhibitors VS-6063 and VS-4718, and the dual mTORC1/2 and PI3K inhibitor VS-5584. We are running clinical trials in cancers where cancer stem cells are implicated in disease progression, including mesothelioma, ovarian, non-small cell lung and other advanced cancers.

Conventional chemotherapy works 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 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.

To enhance therapeutic benefit, we may use our product candidates in combination with other therapies in an effort to target both CSCs and other types of cancer cells. This approach is being utilized in our ongoing ovarian cancer trial of VS-6063 in combination with paclitaxel and our ongoing trial of VS-6063 in combination with VS-5584 for patients with relapsed mesothelioma. A second approach to using CSC-targeting drugs in combination with chemotherapy is based upon the timing (or sequencing) of drug treatment. We believe that using our CSC-targeting drugs immediately following chemotherapeutic treatments (known as maintenance therapy) may complement the overall effect of

treatment by eliminating the residual CSCs following chemotherapeutic treatment. We are using this approach in patients with mesothelioma in our COMMAND trial for VS-6063.

We are developing our product candidates for the treatment of multiple cancer types, initially mesothelioma, ovarian and non-small cell lung cancer, and in the future, other cancers driven by CSCs. We believe that our product candidates target CSCs that have been implicated in aggressive cancers, metastasis and chemotherapeutic resistance.

MESOTHELIOMA

Overview

Malignant mesothelioma is a malignant tumor of mesothelial cells which make up the pleura, or tissue lining, of many internal organs. Mesothelioma most commonly occurs in the pleura surrounding the lung, but occasionally involving the peritoneum surrounding internal organs of the digestive tract, the lining of the heart or the lining of the testis.

Asbestos fibers are the cause of most cases of mesothelioma. The proportion of men with mesothelioma directly attributable to occupational asbestos exposure is approximately 85%. However, mesothelioma can also result from para-occupational exposures (for example, people who have laundered contaminated clothing) and non-industrial environmental exposures. The latency period between first exposure to asbestos and death from mesothelioma is typically very long (20 to more than 40 years).

Mesothelioma is a relatively rare disease; it is estimated that the prevalence within the United States is approximately 2,500-3,000 cases per year and in the European Union it is approximately 8,000 cases per year. The World Health Organization estimates that there are approximately 59,000 cases of mesothelioma each year worldwide.

A large majority of mesothelioma patients present with the pleural form around the lungs. Typically, symptoms are chest pain, shortness of breath, or both. The pain is usually dull, diffuse and characteristically worsens during the course of the illness. The pain may be described as heaviness or aching in the shoulder, arm, chest wall and upper abdomen.

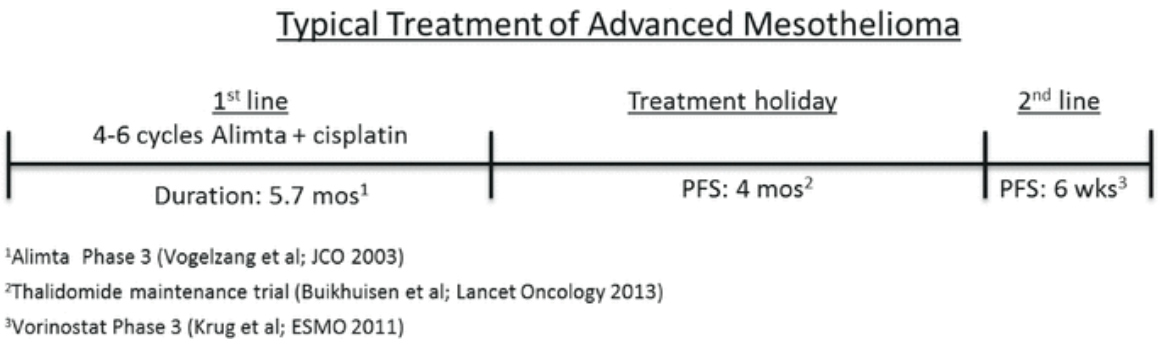
A 2011 study published in Oncogene reported that 90% of mesothelioma patient samples contained CSCs and implicated these cells in the development of drug resistance. In addition, the treatment of mesothelioma cells with pemetrexed (Alimta) led to an increase in the tumor-initiating capability of the drug resistant cells, as compared to those treated with placebo control. A recent report published in the journal Cancer Research describes that the dual loss of merlin and p16 in mesothelioma leads to highly aggressive tumors enriched in cancer stem cells.

Biomarkers

Neurofibromatosis 2 (NF2) is a tumor suppressor gene that encodes the protein merlin. Inactivation of NF2 by mutation and/or deletion, leading to a reduction in the merlin protein, occurs in approximately 40-50% of malignant mesotheliomas. Merlin has been demonstrated to play roles in cell adhesion, invasion and cell motility in tumor cell lines partially through regulation of FAK. In turn, FAK mediates signal transduction by integrins and growth factor receptors. Increased activation of FAK has been demonstrated in merlin-low mesothelioma cells, indicating that FAK may represent an important therapeutic target for malignant mesothelioma. We may develop a diagnostic for merlin to aid the use of VS-6063 in mesothelioma. We believe the patients with tumors that are merlin-low may respond better to treatment with VS-6063.

Current Treatment

Malignant mesothelioma carries a poor prognosis with a median survival of approximately 12 months from diagnosis. Mesothelioma is currently treated with a combination of surgery, radiotherapy and chemotherapy. The only approved drug treatment regimen for mesothelioma is Alimta in combination with carboplatinum or cisplatinum, referred to here collectively as cisplatin. A study by Vogelzang in 2003 reported that the median survival of patients treated with the combination of Alimta and cisplatin was 12.1 months as compared to the median survival of patients treated with cisplatin alone, which was 9.3 months. Following this first-line treatment, the duration of which is often limited by toxicity, patients are monitored but do not receive further therapy until disease progression. As reported by Buikhuisen et al., 2013, the median time to disease progression (progression free survival, or PFS) is approximately 4 months. Once progression occurs, there is no approved standard of care. As reported by Krug et al., in 2011 in an evaluation of 332 patients, this second-line setting has a median PFS of 6 weeks.



OVARIAN CANCER

Overview

Ovarian cancer forms in tissues of the ovary, one of a pair of female reproductive glands in which the ova, or eggs, are formed. Most ovarian cancers are either ovarian epithelial carcinoma, cancer that begins in the cells on the surface of the ovary, or malignant germ cell tumors that begin in egg cells. According to the National Cancer Institute, epithelial carcinoma of the ovary is one of the most common gynecologic malignancies and the fifth most frequent cause of cancer death in women, with 50% of all cases occurring in women older than 65 years.

The National Cancer Institute estimated that in January 2009 there were approximately 182,000 women in the United States with a history of ovarian cancer and in 2013 approximately 22,000 new cases of ovarian cancer would be diagnosed and approximately 14,000 women would die from the disease.

Prognosis

For patients with ovarian cancer, the most important prognostic factor is stage of the disease. Unfortunately, most patients with ovarian cancer have widespread disease at diagnosis. This may be partly explained by relatively early spread to the rest of the abdominal cavity. General symptoms such as abdominal pain and swelling, gastrointestinal symptoms, and pelvic pain often go unrecognized, leading to delays in diagnosis. This is illustrated by the information in the following table, which shows, according to the National Cancer Institute's SEER Cancer Statistics Review, 2004-2010, the reduction in five-year survival rate for ovarian 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.

Stage at Diagnosis	Stage Distribution (%)	5-year Relative Survival (%)
Localized (confined to primary site)	15	92.3
Regional (spread to regional lymph nodes)	18	71.7
Distant (cancer has metastasized)	61	27.4
Unknown (unstaged)	6	21.8

Current Treatment

Most patients are treated with a combination of surgery, chemotherapy, targeted therapy and radiation therapy. Surgery is often comprehensive to remove as much of the tumor as possible and may include removal of the ovaries or a total hysterectomy where the uterus is also removed.

Unfortunately, available therapies are rarely curative in the treatment of ovarian cancer and many tumors become resistant to platinum-based chemotherapy, which is the primary treatment regimen. Further therapy with conventional chemotherapy is generally palliative, not curative, as the tumor is able to metastasize and spread to other sites in the body. A report by McNeish et al., at the 2013 American Society of Clinical Oncology reported that the use of paclitaxel in patients that had previously failed platinum-based chemotherapy resulted in less than 1% of patients achieving a complete response and an overall response rate of approximately 12% as defined by Response Evaluation Criteria In Solid Tumors (RECIST) guidelines. We believe it is the presence of CSCs that lead to this resistance and eventual disease progression.

NON-SMALL CELL LUNG CANCER

Overview

According to the National Cancer Institute, the most common types of non-small cell lung cancer (NSCLC) are squamous cell carcinoma, large cell carcinoma, and adenocarcinoma. Although NSCLCs are associated with cigarette smoke, adenocarcinomas may be found in patients who have never smoked. As a class, NSCLCs are relatively insensitive to chemotherapy and radiation therapy compared with small cell lung cancer (SCLC). The National Cancer Institute estimates that in 2013 there were 228,190 new cases of lung cancer (both NSCLC and SCLC) in the United States and 159,480 deaths. Lung cancer is the leading cause of cancer-related mortality in the United States. The 5-year relative survival rate from 2004 to 2010 for patients with lung cancer was 16.8%.

A study published in Cancer Discovery in 2013 described the role of genetic mutations in several genes that drive carcinogenicity in NSCLC including Kras, p16 and p53. The researchers published data from preclinical models suggesting that tumors harboring both a Kras mutation in addition to a mutation in either p16 or p53 may be more sensitive, as determined by overall survival, to treatment with a FAK inhibitor (VS-6062; an early compound in the same chemical series as VS-6063).

Current Treatment

Patients with resectable disease may be cured by surgery or surgery followed by chemotherapy. Local control can be achieved with radiation therapy in a large number of patients with unresectable disease, but cure is seen only in a small number of patients. Patients with locally advanced unresectable disease may achieve long-term survival with radiation therapy combined with chemotherapy. Patients with advanced metastatic disease may achieve improved survival and palliation of symptoms with chemotherapy, targeted agents, and other supportive measures. The disease becomes resistant to therapy and returns in the vast majority of patients. We believe it is the presence of CSCs that lead to this resistance and eventual disease progression.

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 estimated that in the United States in 2011, approximately 230,500 new cases of invasive breast cancer would be diagnosed in women and approximately 39,500 women would die from the disease.

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

- estrogen receptor, or ER;
- 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 as compared to the original tumor because the CSCs which are not killed are able to metastasize.
- The recurring tumor may be resistant to therapy because it contains a high percentage of CSCs.

Current Treatment

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, Tykerb and Kadcyla. 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, Kadcyla and anti-estrogen treatments, are not effective for these patients. 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.

FAK Inhibition Program

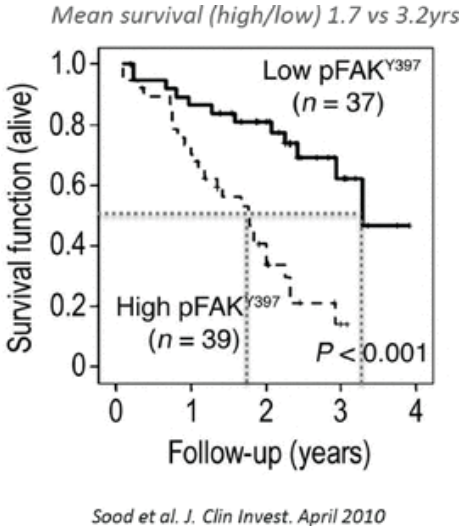
Overview

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 cancer cells and connective tissue stimulates FAK signaling. However, CSCs 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 metastatic growth at other sites in the body.

In 2012, our scientific co-founder Dr. Robert Weinberg reported in Cancer Discovery that FAK is a critical regulator of CSCs and inhibition of this signaling pathway prevented disease progression and initiation of new metastatic lesions. An additional study by Dr. Weinberg reported in PNAS in 2009, demonstrated that in a mouse model of breast cancer FAK signaling was required to enable lung metastasis. Non-CSCs, 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. 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. Similarly, researchers at the University of Michigan demonstrated that specific deletion of FAK from mammary tumors prevented tumor initiating capacity in recipient mice.

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 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. A similar finding was described in the Journal of Clinical Investigation in 2010 where it was reported that in women with ovarian cancer elevated FAK expression is correlated with advanced disease, metastases and poor prognosis. A comparison of FAK activity in biopsies from

patients revealed that high FAK activity correlated with a decrease in overall survival, from 3.2 to 1.7 years.



Sood et al. J. Clin Invest. April 2010

VS-6063

Overview

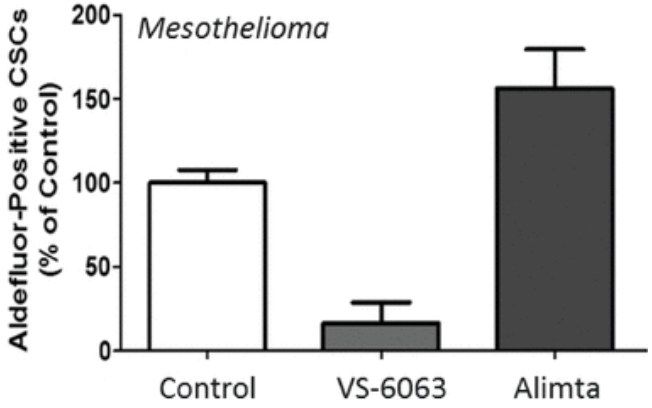
VS-6063, or defactinib, is an orally-available small molecule compound designed to target cancer stem cells through the inhibition of FAK signaling. We are currently evaluating VS-6063 as a potential therapy for mesothelioma, ovarian cancer, NSCLC and other solid tumors driven by CSCs. We are currently conducting clinical trials of VS-6063 in patients with cancer: including the registration-directed COMMAND study in patients with mesothelioma, a Phase 1/1b clinical trial of VS-6063 in combination with paclitaxel for patients with ovarian cancer, a Phase 2 study of VS-6063 in patients with NSCLC, a Phase 2 trial preceding surgery in mesothelioma and a Phase 1 combination study of VS-6063 and VS-5584 in patients with relapsed mesothelioma. We have received orphan drug designation for VS-6063 in mesothelioma in both the United States and the European Union.

Development status of VS-6063

Registration-directed study in mesothelioma (COMMAND). We are conducting a randomized, double-blind, multi-center, multi-national study of VS-6063 in patients with mesothelioma and at least stable disease post first-line therapy. We have met with the regulatory agencies in the United States, the United Kingdom and Japan and interacted with the regulatory agencies in ten other countries to discuss COMMAND and, based on these discussions, we believe that positive results, if obtained, from this trial may enable us to seek regulatory approval for VS-6063 in mesothelioma. The study is currently open and recruiting patients in 13 countries at 55 study centers. We have enrolled 180 patients as of January 8, 2015, and in total, we expect to enroll 350-400 patients into COMMAND and to complete enrollment by the end of 2015.

Even when front line therapy of Alimta plus cisplatin produces an objective response or stable disease, patients are noted to have progression of disease within approximately four months of completing therapy. The ability to maintain the responses seen with first line therapy and delay further progression is therefore a critical goal in this disease. We believe it is the presence of chemotherapy-resistant CSCs in the residual tumor that lead to progression and it is our goal to extend the amount of disease control time for patients through treatment with VS-6063.

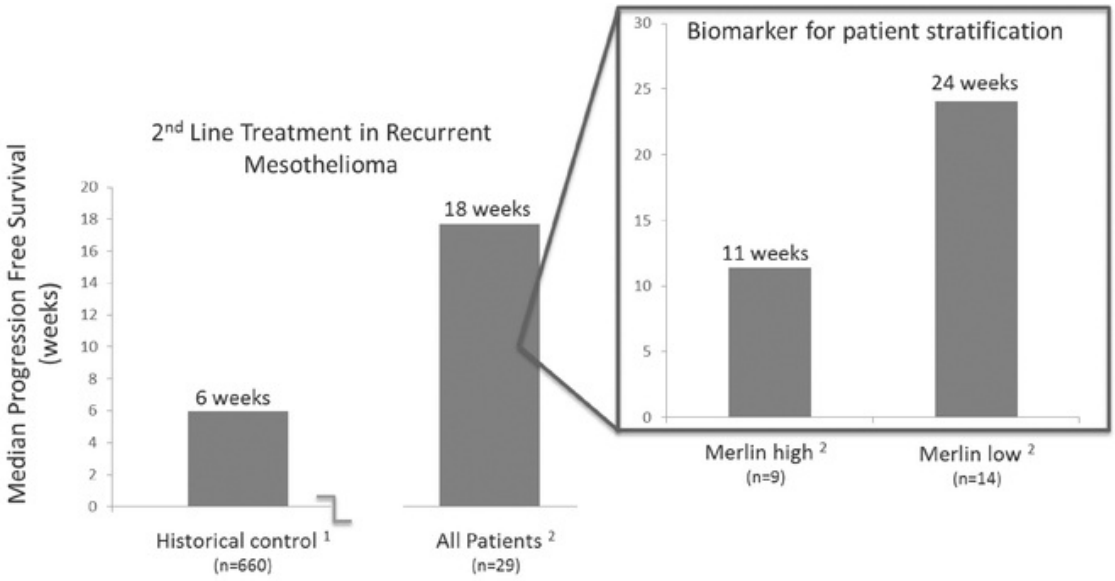
Researchers hypothesized in 2010 in the International Journal of Oncology that early relapse of mesothelioma and further progression following Alimta plus cisplatin results from the presence of a relatively small number of CSCs which exhibit resistance to standard therapy. This hypothesis is supported by our experiments with human mesothelioma cell lines which show that chemotherapeutic agents such as Alimta and cisplatin enrich for CSCs. As shown below, in contrast to Alimta, inhibition of FAK by VS-6063 effectively reduces the proportion of CSCs, as determined by the biomarker Aldefluor. This suggests that targeting FAK might be expected to reduce the CSC population and overcome the resistance that develops to Alimta and cisplatin in mesothelioma.



We believe the sequential dosing of chemotherapy (eg. Alimta plus cisplatin) and a CSC-targeted agent (VS-6063) in the maintenance setting immediately following front-line therapy will allow us to treat both the non-CSCs and CSCs.

In a report at the NCI-AACR-EORTC conference in 2012, in a Phase 1 study of the orally available FAK inhibitor GSK2256098 by GlaxoSmithKline (GSK), a median PFS of 17.7 weeks was observed in 29 patients with recurrent mesothelioma in comparison to the previously reported median PFS of just 6.1 weeks in 332 similar patients receiving placebo. In addition, GSK evaluated the response to treatment based upon the status of the biomarker merlin. Patients who were deemed to be

merlin-low had a median PFS of 24.1 weeks compared with 11.4 weeks for those patients whose tumors were deemed to be merlin-high as shown in the figure below.



[1] Historical data from Vorinostat Phase 3 (Krug et al; ESMO 2011)

[2] Phase 1 trial of GSK2256098 presented at EORTC-NCI-AACR Molecular Therapeutics meeting (Nov. 6-9, 2012)

These observations generally suggest that FAK inhibition in mesothelioma may be helpful to slow disease progression and may indicate that use of a diagnostic to identify patients with low levels of the biomarker merlin may predict for those most likely to have the greatest response to treatment with a FAK inhibitor.

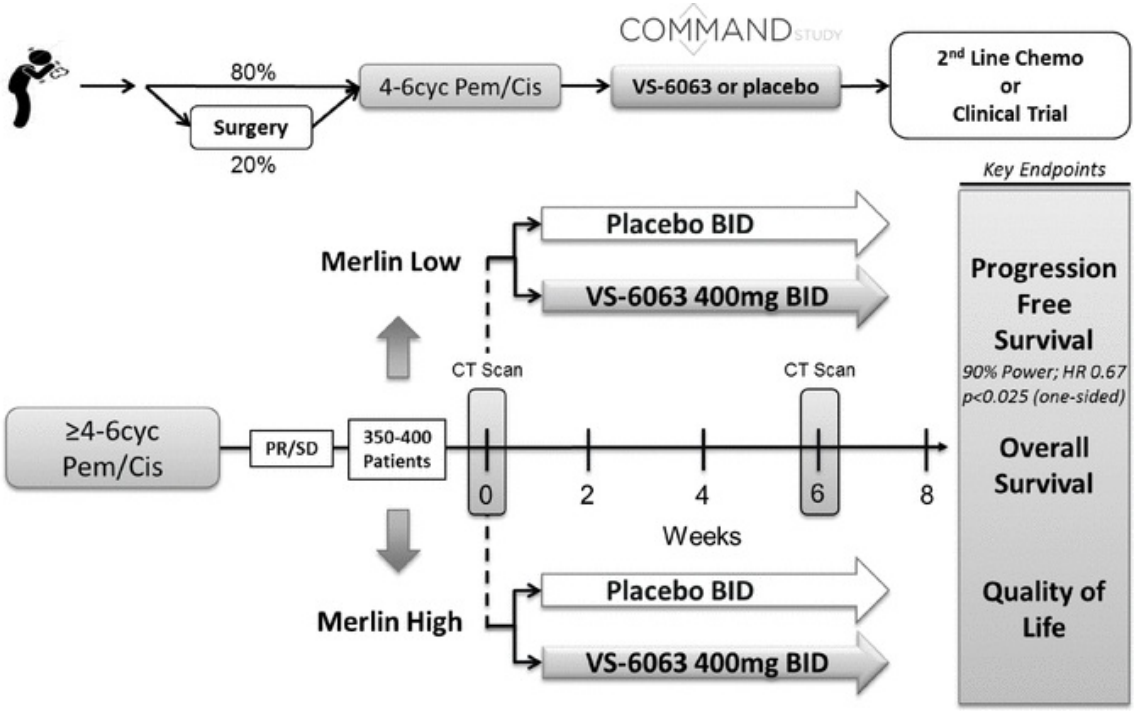
COMMAND is recruiting patients who have not progressed on at least 4 cycles of Alimta plus cisplatin as their most recent therapy. Immediately following completion of the front-line chemotherapy, patients begin to take VS-6063 or placebo by mouth, twice a day. This treatment strategy is known as switch maintenance therapy and there is no current standard of care recommended for patients with mesothelioma in this setting. The patients are stratified by their merlin expression, as described below, and within each stratum are then randomized to either VS-6063 or matched placebo.

Mesothelioma is a rare disease making it important to derive as much information as possible, with as great a certainty as possible, from each clinical trial. It is hypothesized that the treatment effect in the subjects with merlin low tumors is likely to be greater than that in the merlin high group. Therefore, our design incorporates a sample size reassessment at a pre-planned interim analysis. The goals of the enrichment design approach are:

- To identify if the overall population is benefitting or if the merlin low sub population is responding better to treatment.
- To modify patient enrollment and only continue enrolling patients with merlin low tumors if those patients are responding better to treatment.

This study design enables an analysis that could support approval in either the full treatable mesothelioma population or the approximately half of the patients with merlin low tumors. We believe the use of a companion diagnostic test to select the patients with merlin low tumors who may respond

better to FAK treatment in mesothelioma may increase our ability to properly treat this patient population if the COMMAND trial is adapted to enrich for patients with merlin tumors.

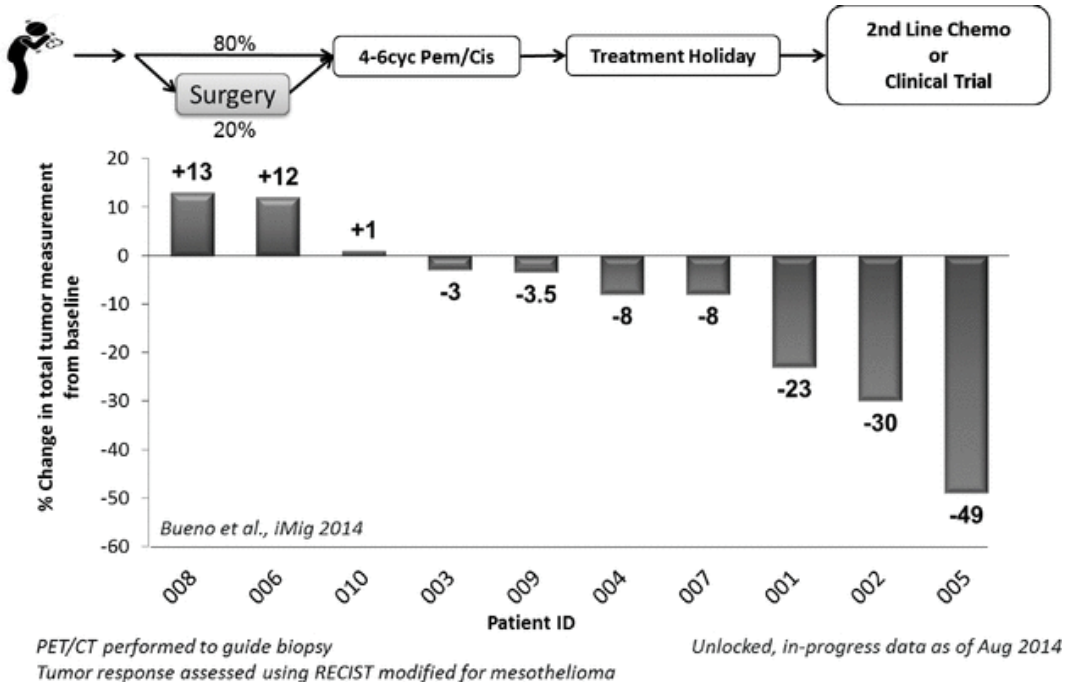


The key endpoints of the study are overall survival, progression free survival and quality of life. Based on discussions with the regulatory agencies in the United States, United Kingdom and Japan and interactions with regulatory agencies in ten other countries, we believe that if VS-6063 provides significant clinical benefit, we may be able to seek regulatory approval.

Phase 2 trial preceding surgery in mesothelioma (Window of Opportunity). VS-6063 is currently in an open-label neoadjuvant study in subjects with malignant pleural mesothelioma who are eligible for surgery. Neoadjuvant refers to the administration of VS-6063 prior to surgery to remove the mesothelioma tumor. The purpose of this study is to assess biomarker responses from tumor tissue. The study is being conducted in two parts. In part one, 10 subjects were administered VS-6063 for 12 days. In part two, 10-15 subjects will receive VS-6063 for 35 days. Pre- and post-treatment biopsies and blood samples are being collected and PET/CT scans are being performed both pre- and post-treatment. The safety, pharmacokinetics, and tumor response rate to VS-6063 are also being assessed.

In October 2014, results from part one were presented at the International Mesothelioma Interest Group (iMig) meeting. Based upon biopsy results, treatment with VS-6063 for 12 days reduced FAK activity by an average of 70% in evaluable biopsies and decreases in a marker of cancer stem cells were observed in five of seven evaluable biopsies. In addition, the results of the pre- and post-treatment PET/CT scans, as measured by Response Evaluation Criteria in Solid Tumors, or RECIST, modified for mesothelioma, indicated that no disease progression was observed and that reductions in tumor size consistent with a partial response were observed in two patients. A waterfall plot of the tumor size

measurements is presented in Figure 8. Part two of the study, where the treatment period is increased from 12 to 35 days, is currently ongoing.



Phase 2 study of VS-6063 in patients with non-small cell lung cancer (NSCLC). We are conducting a multi-center study of VS-6063 in patients with recurrent NSCLC following previous chemotherapy. There are 4 study arms, including patients with a KRAS-mutation, or a KRAS-mutation with accompanying secondary mutations in p16, p53 or both p16 and p53. The trial is being conducted at 9 sites in the US and is expected to enroll up to 44 eligible patients (11 per arm) in the first stage of the Simon's two-stage trial design. A Simon's two-stage trial design incorporates a single pre- planned interim analysis to determine which enrollment arms are exhibiting a favorable response to treatment and allows for enrollment of an additional 12 patients with the same tumor mutation profile per arm. The Phase 2 study is designed to assess the effect of VS-6063 on progression free survival, overall response rate, and overall survival.

As of January 8, 2015, the two cohorts that had completed enrollment of stage one, each had reached the interim threshold of greater than or equal to 4 patients achieving disease control for greater than or equal to 12 weeks. We have stopped accrual to the study and are evaluating designs of potential follow up trials.

Phase 1/1b clinical trial of VS-6063 in combination with paclitaxel in patients with ovarian cancers. Ovarian cancer rapidly develops resistance to chemotherapy. Following development of resistance to front-line platinum therapy, there is not an approved standard of care although treatment with the chemotherapeutic paclitaxel is often used. We are currently conducting a clinical trial to evaluate the safety and efficacy of VS-6063 administered in combination with paclitaxel in patients with advanced ovarian cancer.

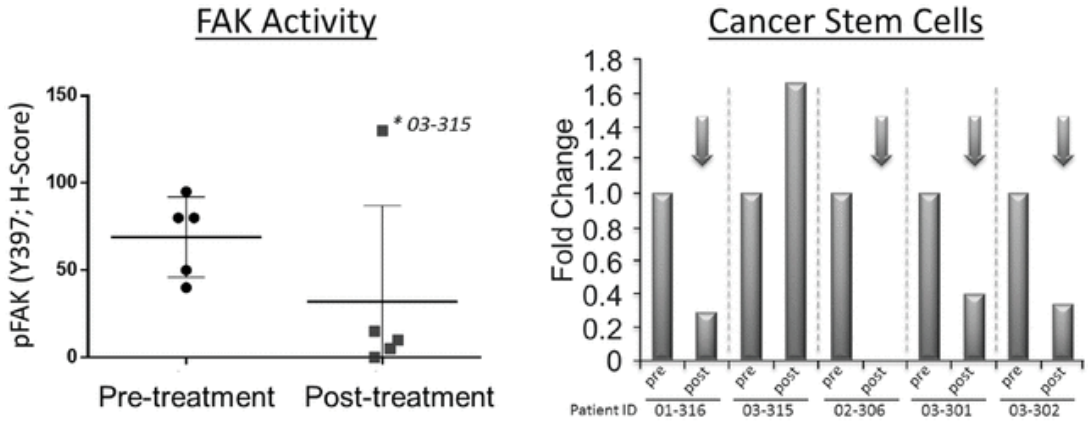
This clinical study is comprised of two parts. Part 1 is a dose escalation of oral VS-6063 twice daily in addition to the standard dose of weekly paclitaxel. The purpose of part 1 is to assess the safety, evaluate pharmacokinetic parameters and determine the recommended Phase 2 dose in combination

with paclitaxel. Part 2 includes patients treated with the recommended Phase 2 dose of VS-6063 in combination with paclitaxel. The purpose of part 2 is to evaluate the pharmacodynamic effect, the biochemical and physiological effect of VS-6063 through measurement of biomarkers from tumor biopsies pre- and post-treatment and assess the anti-cancer activity of VS-6063 when administered in combination with paclitaxel according to RECIST guidelines.

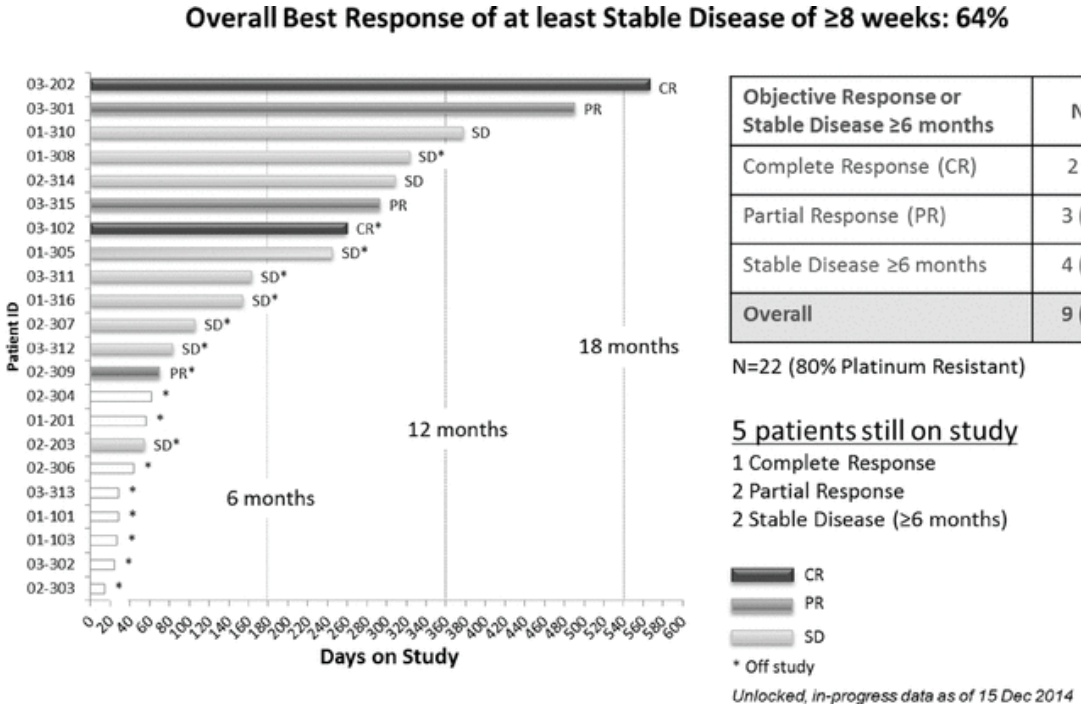
We believe this approach will allow us to treat both the non-CSCs and CSCs simultaneously by the combination of chemotherapy (eg. paclitaxel) and a CSC-targeted agent (VS-6063) to hopefully enable a more durable clinical response.

As of June 30, 2014, seventeen serious adverse events, or SAEs, were observed in 9 patients (41%). All but one SAE (Grade 3 dehydration, deemed possibly related) were deemed to be not related or unlikely. In general, the addition of VS-6063 to weekly paclitaxel was generally well tolerated with no worsening of the well-known side effects of paclitaxel at either the 200mg/BID or 400 mg/BID dose level of VS-6063 in addition to the standard dose of weekly paclitaxel of 80mg/m2. 400mg/BID is the recommended Phase 2 dose of VS-6063 both as a single agent and in combination with weekly paclitaxel.

As of December 15, 2014, 64% of patients have achieved a best overall response of at least stable disease of greater than or equal to 8 weeks as per RECIST guidelines in this ongoing study. Biomarker data on biopsies obtained pre- and post-treatment with VS-6063 (400mg BID) as a single agent for 10 days shows reductions in FAK activity and decreases in cancer stem cells. In addition, of the twenty-two patients treated with the combination of VS-6063 and paclitaxel, three partial responses and two complete responses have been reported as of December 15, 2014. Five patients remained on study as of December 15, 2014.



Paired tumor biopsies were obtained in five ovarian patients following 10 days of VS-6063 administration (400 mg BID)



Objective Response or Stable Disease ≥6 months	N (%)
Complete Response (CR)	2 (9%)
Partial Response (PR)	3 (14%)
Stable Disease ≥6 months	4 (18%)
Overall	**9 (41%)**

N=22 (80% Platinum Resistant)

5 patients still on study
1 Complete Response
2 Partial Response
2 Stable Disease (≥6 months)

CR
PR
SD
* Off study

Unlocked, in-progress data as of 15 Dec 2014

Phase 1 clinical trial in advanced solid tumors. VS-6063 demonstrated a good safety profile and initial signs of clinical activity in a Phase 1 trial in 46 patients with advanced solid tumors. Patients were selected on the basis of a confirmed diagnosis of a non-hematologic malignancy that was unresponsive to currently available therapies or for which no standard therapy existed. VS-6063 was administered on a twice daily (BID) schedule and dose cohorts initially included a minimum of 3 patients. Dosing was based on flat milligram increments without adjustment for body size, the starting dose being 12.5 mg BID in the initial cohort. Safety, activity and pharmacokinetics, the process by which a drug is absorbed, distributed and metabolized in the body, were measured. 16 of the 37 subjects enrolled, or 43%, at doses greater than or equal to 100 mg BID (predicted active dose) experienced stable disease as their best response to treatment. The recommended Phase 2 dose based on safety, pharmacokinetics and activity was determined to be 400 mg/BID.

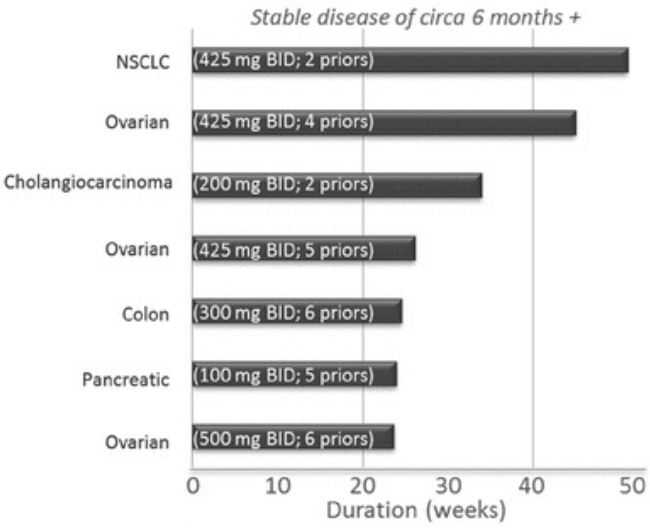
Generally Well Tolerated

Adverse Events*	Grade 1 N (%)	Grade 2 N (%)	Grade 3 N (%)	Grade 4 N (%)	Total N (%)
Nausea	14 (30)	3 (7)	0	0	17 (37)
Unconjugated hyperbilirubinemia	6 (13)	9 (20)	2 (4)	0	17 (37)
Fatigue	8 (17)	6 (13)	1 (2)	0	15 (33)
Vomiting	10 (22)	3 (7)	0	0	13 (28)
Headache	9 (20)	0	1 (2)	0	10 (22)
Diarrhea	8 (17)	2 (4)	0	0	10 (22)
Decreased appetite	8 (17)	1 (2)	0	0	9 (20)

*Treatment-Related Adverse Events (≥20%)

Jones SF J Clin Oncol 2011 29:1 (suppl; abstr 3002)

Initial Signs of Clinical Activity



Phase 1 study of VS-6063 in Japanese patients. In November 2014, we reported data from a Phase 1 dose escalation study of VS-6063 as a single agent in Japanese subjects. The dose levels were 200, 400 or 600mg of VS-6063 BID. No dose limiting toxicities, or DLTs, or SAEs were observed at any dose cohort. All treatment emergent adverse events were generally mild or moderate in nature, or grade 1 or 2 as determined in accordance with commonly accepted terminology for adverse events, except for one subject in the 200mg BID cohort who exhibited a Grade 3 increase in serum bilirubin, defined as between 3 and 10 times the value of the upper limit of the normal laboratory range. No associated increase in liver enzymes, AST or ALT, were observed in this subject.

The study results showed that VS-6063 was generally well tolerated and side effects were consistent with previously reported results from our U.S. Phase 1 trial. One patient in the study had mesothelioma which had relapsed following frontline chemotherapy and was on VS-6063 for 5.6 months with best response of stable disease.

Adverse Event	200 mg defactinib BID (n=3)	400 mg defactinib BID (n=3)	600 mg defactinib BID (n=3)	Total (n=9)
Blood Bilirubin Increased	3 (100.0%)	2 (66.7%)	2 (66.7%)	7 (77.8%)
Fatigue	2 (66.7%)	1 (33.3%)	3 (100.0%)	6 (66.7%)
Decreased Appetite	2 (66.7%)	1 (33.3%)	1 (33.3%)	4 (44.4%)
Diarrhoea	0 (0.0%)	1 (33.3%)	2 (66.7%)	3 (33.3%)
Anaemia	0 (0.0%)	0 (0.0%)	2 (66.7%)	2 (22.2%)
AST Increased	1 (33.3%)	0 (0.0%)	1 (33.3%)	2 (22.2%)
Blood Alk Phos Increased	2 (66.7%)	0 (0.0%)	0 (0.0%)	2 (22.2%)
Cancer Pain	1 (33.3%)	1 (33.3%)	0 (0.0%)	2 (22.2%)
Headache	0 (0.0%)	1 (33.3%)	1 (33.3%)	2 (22.2%)
Nausea	1 (33.3%)	0 (0.0%)	1 (33.3%)	2 (22.2%)

VS-4718

Overview

VS-4718 is an orally-available small molecule compound designed to target cancer stem cells through the inhibition of FAK signaling. We are evaluating VS-4718 as a potential therapy for cancers driven by CSCs.

Development status of VS-4718

We are conducting a Phase 1, dose escalation clinical trial of our FAK inhibitor, VS-4718, in patients with advanced cancers. As of January 8, 2015, the Maximum Tolerated Dose (MTD) had not been reached. Three patients on the study have mesothelioma and two of these mesothelioma patients have had disease stabilization for at least five months.

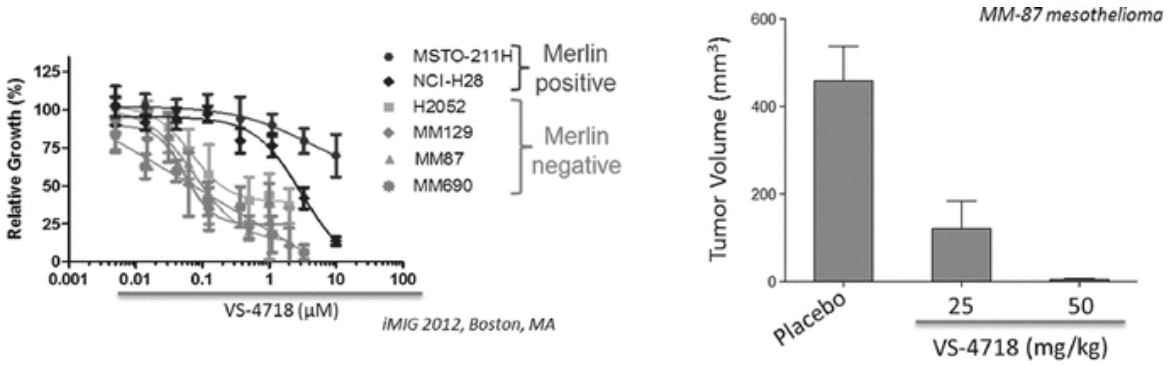
Preclinical development

We are conducting a comprehensive program to study VS-4718 as a potential treatment for cancers associated with CSCs and increased FAK activity. Key results to date from preclinical studies of VS-4718 are summarized below.

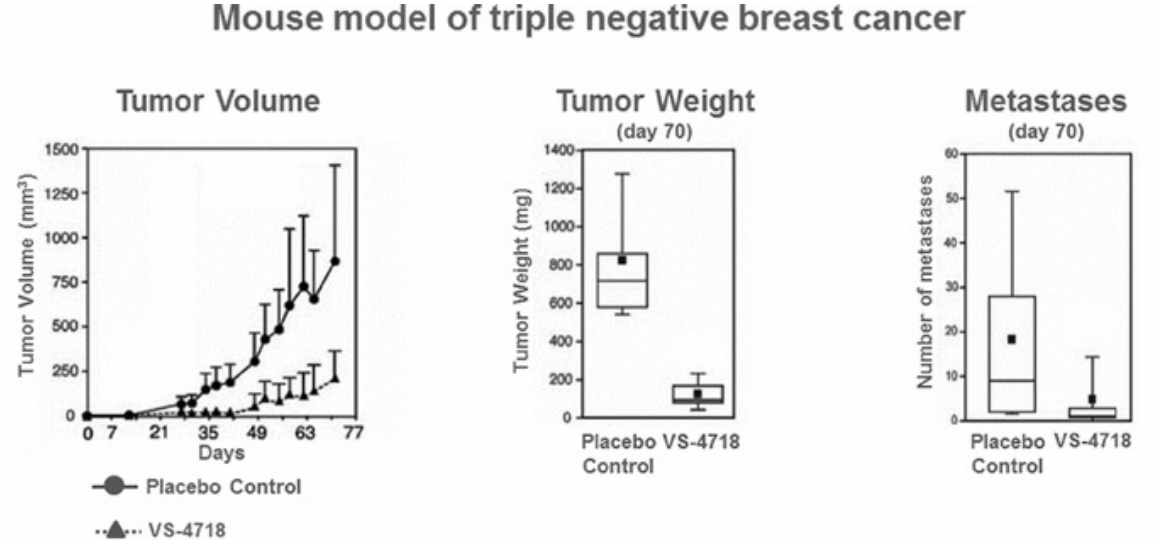
Biochemical and cellular tests. In biochemical testing, VS-4718 inhibited purified FAK and demonstrated *in vitro* selectivity against a panel of 107 different protein kinases. In addition, in various *in vitro* assessments of drug effects on CSCs, VS-4718 exhibited potent activity and a preferential effect, or selectivity, for CSCs as compared to other types of cancer cells.

Effect of FAK inhibition in merlin low cell lines and mouse tumor models of mesothelioma. VS-4718 has exhibited in vitro cytotoxic effects on mesothelioma cell lines and tumor growth inhibition in mouse models of mesothelioma. Merlin low cells lines derived from mesothelioma tumors were observed to be

particularly sensitive to FAK inhibition. VS-4718 was also tested in models in which merlin low mesothelioma cells were implanted into a mouse and the tumor was allowed to develop. Upon tumor formation, the mice were treated with VS-4718 by oral administration at concentrations of 25 or 50 mg/kg of body weight or a placebo control through the end of the experiment. As shown in the figure below, single agent VS-4718 treatment caused tumor regression in a dose-dependent fashion in contrast to placebo control.



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 breast cancer cells were implanted into a mouse and the tumor was allowed to develop. Upon tumor formation, the 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.

PI3K/mTOR Inhibition Program

Overview

The PI3 kinase/mTOR pathway plays a central role in cancer cell proliferation and survival. Depending on the mode of pathway activation, different PI3K isoforms and mTOR complexes have been shown to play essential roles in oncogenesis and survival of CSCs. There are four Class 1 PI3K enzymes, known as alpha, beta, gamma and delta, and two mTOR complexes known as mTORC1 and mTORC2.

Studies published in Nature in 2006 reported that genetic activation of the PI3K/mTOR pathway in adult blood cells led to generation of leukemia CSCs. In addition, multiple studies have shown that PI3K pathway activity contributes to the maintenance of CSCs in breast, lung, and prostate cancers.

VS-5584

Overview

VS-5584 is an orally available small molecule that potently and selectively inhibits both mTORC1/2 and PI3K signaling. A 2013 study published in Science Translational Medicine described findings that the observed resistance to PI3K- alpha inhibitors in breast cancer is mediated by signaling through the mTOR complex. We believe that the dual inhibition of mTORC1/2 and PI3K by VS-5584 may provide a greater clinical benefit for patients.

Development status of VS-5584

We are conducting a Phase 1, dose escalation clinical trial of our dual mTORC1/2 and PI3K inhibitor, VS-5584, in patients with advanced cancers. As of January 8, 2015, the MTD had not been reached. Reductions in pharmacodynamic markers of PI3K and mTOR activity have been observed. Clinical activity has been observed in mesothelioma and other tumors. Disease control of at least six months has been observed in some patients. We have received orphan medicinal product designation from the European Commission for the use of VS-5584 in the treatment of mesothelioma. A Phase 1 clinical trial of VS-5584 in combination with VS-6063 for patients with relapsed mesothelioma is also currently ongoing.

Preclinical development

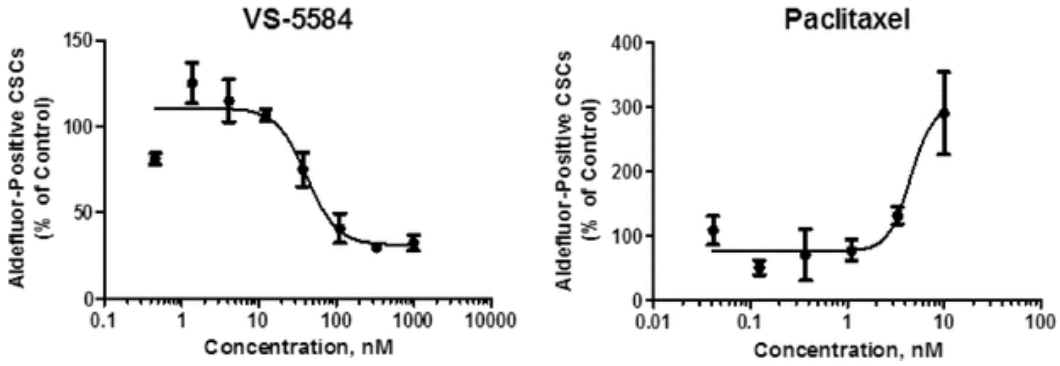
We are conducting a comprehensive preclinical program to study VS-5584 as a potential treatment for cancer. Detailed results from this development were published in the January 2015 edition of the journal *Cancer Research*. Key results of this program to date are summarized below.

Biochemical testing. In biochemical testing, VS-5584 was equipotent against all four human Class 1 PI3K isoforms and mTOR kinase, the enzymatic component of both mTORC1 and mTORC2 (IC_{50} values displayed below in nM). In general, the more potent a drug is, the lower the dose required for a therapeutic effect. An IC_{50} value measures the dose at which the activity of the enzyme is reduced

by 50%, a commonly used measure of potency. VS-5584 has demonstrated a selective effect for these kinases versus a panel of over 400 kinases.

mTOR	Alpha	Beta	Delta	Gamma
3.4	2.6	21	3.0	2.7

In vitro killing of CSCs. In an in vitro test, SUM159 triple negative breast cancer cells were treated with VS-5584 or paclitaxel for 4 days, and the percentage of Aldefluor-positive CSCs was assessed. VS-5584 dose-dependently decreased the percentage of CSCs, while paclitaxel increased the percentage of CSCs.



INTELLECTUAL PROPERTY

We strive to protect the proprietary technology that we believe is important to our business, including seeking and maintaining patents intended to cover our product candidates and compositions, their methods of use and processes for their manufacture, and any other aspects of 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.

We plan to continue to expand our intellectual property estate by filing patent applications directed to compositions, methods of treatment and patient selection created or identified from our ongoing development of our product candidates. 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.

The patent positions of biopharmaceutical companies like us are generally uncertain and involve complex legal, scientific and factual questions. In addition, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and patent scope can be reinterpreted by the courts after issuance. Moreover, many jurisdictions permit third parties to challenge issued patents in administrative proceedings, which may result in further narrowing or even cancellation of patent claims. We cannot predict whether the patent applications we are currently pursuing will issue as patents in any particular jurisdiction or whether the claims of any issued patents will provide sufficient protection from competitors.

Because patent applications in the United States and certain other jurisdictions are maintained in secrecy for 18 months or potentially even longer, and since publication of discoveries in the scientific or patent literature often lags behind actual discoveries, we cannot be certain of the priority of inventions covered by pending patent applications. Moreover, we may have to participate in interference proceedings or derivation proceedings declared by the United States Patent and Trademark Office to determine priority of invention.

Patents

Our patent portfolio includes issued and pending applications worldwide. These patent applications fall into four categories: (1) FAK inhibitor program; (2) PI3K/mTOR inhibitor program; (3) Wnt inhibitor program; and (4) other programs.

FAK inhibitor program

We are currently developing the following FAK inhibitors: VS-6063 and VS-4718.

VS-6063

We have exclusively licensed a portfolio of patent applications owned by Pfizer, which are directed to FAK inhibitor compounds and methods of their use, for example in cancer. One patent family is related generally to VS-6063. This patent family includes issued patents having claims covering VS-6063 generically and specifically. For example, US 7,928,109 covers the composition of matter of VS-6063 specifically and US 8,247,411 covers the composition of matter of VS-6063 generically. Also included are issued and pending patent applications having claims directed to methods of treatment and methods of making VS-6063. For example, US 8,440,822 covers methods of making VS-6063. Any U.S. patents that have issued or will issue in this family will have a statutory expiration date in April of 2028. Related cases are pending worldwide, including for example in Europe, Brazil, Thailand, Hong Kong, and India, and granted in Australia, Mexico, Canada, China, Korea, Israel, New Zealand, South Africa, Singapore, Taiwan, and Japan.

In addition to the issued and pending patent applications exclusively licensed from Pfizer, we own two patent families covering VS-6063. One family is directed to compositions (e.g., oral dosage forms) of VS-6063 and certain methods of use. Any U.S. patents that will issue in this family will have a statutory expiration date in January of 2035. Another family is directed to methods of using a FAK inhibitor, such as VS-6063 in combination with a MEK inhibitor for treating a subject. Any U.S. patents that will issue in this family will have a statutory expiration date in February of 2035.

Our licensed portfolio of patent applications from Pfizer also includes four families of patent applications directed to VS-6062 and related methods of use. The patent families include issued and pending patent applications having claims directed to VS-6062, methods of manufacture, and pharmaceutical salts. Patents have issued in these families in the U.S. that will expire in the December of 2023, April of 2025, and November of 2028, respectively. Related cases have been granted worldwide, including for example in Australia, Canada, China, Japan, and Europe.

VS-4718

We have exclusively licensed a family of patent applications owned by the Scripps Research Institute, which is directed to VS-4718 and related methods of use. For example, US 8,501,763 covers the composition of matter of VS-4718. The statutory expiration of any patent that has issued or will issue pertaining to VS-4718 has or will have a statutory expiration date in March of 2028. Related cases are pending worldwide, including for example in China and Canada. The patent has also been allowed in Japan and Europe.

We also own two families of patent applications directed to VS-4718 formulations and methods of manufacture. Any patents that will issue in either of these families will have a statutory expiration date in June of 2031.

PI3K/mTOR inhibitor program

We own, through acquisition from S*Bio, a portfolio of patent applications covering VS-5584 and related methods of use, including two families of patent applications. One family covers VS-5584 generically and has a statutory expiration date in October of 2028. For example, US 8,247,410 covers the composition of matter of VS-5584 generically and US 8,609,838 covers methods of synthesis of VS-5584. The other family covers VS-5584 specifically. In the U.S., the composition of matter of VS-5584 is covered specifically in US 8,754,080 and will expire at the same time as the first family. Also included are issued and pending patent applications having claims directed to methods of treatment and methods of making VS-5584. A related case in Europe has also been allowed and been validated in Germany, Denmark, Spain, Finland, France, the United Kingdom, Ireland, Italy, the Netherlands, and Sweden.

In addition, we own one patent family directed to a clinical dosing protocol for VS-5584. Any patents that will issue in this family will have a statutory expiration date in December of 2035.

Wnt inhibitor program

We have exclusively licensed a portfolio of patent applications owned by Broad, Whitehead and Harvard. The family also 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, for example, based on the presence of a biomarker. Any U.S. or EU patents that may issue from this application will have a statutory expiration date in April of 2029.

Other

We own or have licenses on five families of patent applications directed to methods of selecting patients and predicting patient response.We own two patent families, both of which have a statutory expiration date in March of 2033. We license three families, which have statutory expirations of March of 2025, June of 2026, and August of 2031. We also exclusively license a patent application that includes claims covering: 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 this application would have a statutory expiration date in 2026.

Patent Term

The base term of a United States patent is 20 years from the filing date of the earliest-filed non-provisional patent application from which the patent claims priority. The term of a United States patent can be lengthened by patent term adjustment, which compensates the owner of the patent for administrative delays at the USPTO. In some cases, the term of a United States patent is shortened by terminal disclaimer that reduces its term to that of an earlier-expiring patent.

The term of a United States patent may be eligible for patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Act, to account for at least some of the time the drug is under development and regulatory review after the patent is granted. With regard to a drug for which FDA approval is the first permitted marketing of the

active ingredient, the Hatch-Waxman Act allows for extension of the term of one United States patent that includes at least one claim covering the composition of matter of an FDA-approved drug, an FDA-approved method of treatment using the drug, and/or a method of manufacturing the FDA-approved drug. The extended patent term cannot exceed the shorter of five years beyond the non-extended expiration of the patent or 14 years from the date of the DFA approval of the drug. Some foreign jurisdictions, including Europe and Japan, have analogous patent term extension provisions, which allow for extension of the term of a patent that covers a drug approved by the applicable foreign regulator agency. In the future, if and when our pharmaceutical products receive FDA approval, we expect to apply for patent term extension on patents covering those products, their methods of use, and/or methods of manufacture.

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, both on its own behalf and as sole and exclusive agent of Harvard and MIT, or the drug discovery platform license agreement, which we amended and restated in January 2012. 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 for use in treating cancer, and to develop and perform licensed processes, in each case, for all human therapeutic, prognostic and diagnostic uses.

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 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 Epithelial to Mesenchymal Transition, or EMT.

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.

Under the agreement, we paid the Whitehead Institute upfront license fees and reimbursed 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 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.

The Scripps Research Institute

In November 2011, we entered into a license agreement with Poniard Pharmaceuticals, Inc., or 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. These licensed patent rights include patent rights owned by The Scripps Research Institute, or Scripps, and licensed to Poniard. Under the agreement, we paid Poniard an upfront license fee and agreed to pay Poniard milestone payments upon the achievement of specified development and regulatory milestones.

On August 2, 2013, patents and other rights which were the subject of our license agreement with Poniard were sold to Encarta, Inc., or Encarta. We purchased these assets from Encarta in an asset purchase agreement dated February 21, 2014 and also entered into a securities issuance agreement. Under the terms of these agreements, we issued 97,500 shares of common stock, issued a warrant to purchase 142,857 shares of common stock with an exercise price equal to $17.16 per share and paid Encarta $25,000. All existing obligations under the license agreement, including an achieved development milestone and an obligation to issue a warrant, were settled as part of this transaction.

In connection with the asset purchase agreement, we also assumed the rights and obligations under the license agreement by and between Scripps and Poniard, dated May 5, 2008, or the Scripps License Agreement. Pursuant to the Scripps License Agreement, we acquired an exclusive, worldwide license under patent rights owned or controlled by Scripps to make and have made, to use and have used, to offer to sell, to sell and have sold, and import products covered by the licensed patent rights for the diagnosis, treatment or prevention of human diseases or conditions. The licensed patent rights include patents covering our product candidate VS-4718. Under the Scripps License Agreement, Scripps retains the right to grant non-exclusive licenses to nonprofit or academic institutions, without the right to sublicense, to use any of the licensed patent rights for any noncommercial research or education purposes.

Pursuant to the Scripps License Agreement, we are obligated to pay Scripps potential product development milestone payments of up to an aggregate of $3,000,000 upon the achievement of specified development and regulatory milestones. In addition, we are obligated to pay Scripps low single-digit royalties as a percentage of net sales of licensed products. Our obligation to pay royalties on net sales is on a country by country basis. In the event that we challenge a patent or patent application covered by the Scripps License Agreement, our royalties will increase by fifty percent during the pendency of the challenge (and increase by one hundred percent in the event the challenge is not successful). We also forfeit the right to recoup any royalties, sublicense payments, milestone payments, patent costs or other payments during the period of any challenge to the patents covered under the Scripps License Agreement.

If we license or acquire technology from a third party in order to commercialize a licensed product and to pay such third party royalties or other amounts, then we may deduct up to 50% of the amount paid to such third party from the payments owed to Scripps 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 Scripps to less than 50% of the otherwise applicable amount.

We are required to use reasonable and diligent efforts to commercialize (directly or through sublicense arrangements) licensed products (including our product candidate VS-4718) in either the United States, the United Kingdom, France, Germany or Japan.

The Scripps License Agreement expires upon the last expiration of any of the licensed patent rights. We have the right to terminate the Scripps License Agreement or any portion of our licensed rights under the Scripps License Agreement for any reason upon at least 90 days prior written notice and payment of a low five-figure termination fee. We are not responsible for the termination fee if Scripps defaults in the performance of its material obligations and fails to cure. Scripps can terminate the Scripps License Agreement for certain material breaches by us or defaults in our performance of material obligations.

Pfizer, Inc.

On July 11, 2012, we entered into a license agreement with Pfizer, Inc., or Pfizer, under which Pfizer granted us worldwide, exclusive rights to research, develop, manufacture and commercialize products containing certain of Pfizer's inhibitors of focal adhesion kinase, including VS-6063, for all therapeutic, diagnostic and prophylactic uses in humans. We have the right to grant sublicenses under

the foregoing licensed rights, subject to certain restrictions. We are solely responsible, at our own expense, for the clinical development of these products, which is to be conducted in accordance with an agreed-upon development plan. We are also responsible for all manufacturing and commercialization activities at our own expense. Pfizer provided us with an initial quantity of clinical supplies of one of the products for an agreed upon price.

Upon entering into the license agreement, we made a one-time cash payment to Pfizer in the amount of $1.5 million and issued 192,012 shares of our common stock. Pfizer is also eligible to receive up to $2 million in developmental milestones and up to an additional $125 million based on the successful attainment of regulatory and commercial sales milestones. Pfizer is also eligible to receive high single to mid double digit royalties on future net sales of the products. Our royalty obligations with respect to each product in each country begin on the date of first commercial sale of the product in that country, and end on the later of 10 years after the date of first commercial sale of the product in that country or the date of expiration or abandonment of the last claim contained in any issued patent or patent application licensed by Pfizer to us that covers the product in that country.

The license agreement will remain in effect until the expiration of all of our royalty obligations to Pfizer, determined on a product-by-product and country-by-country basis. So long as we are not in breach of the license agreement, we have the right to terminate the license agreement at will on a product-by-product and country-by-country basis, or in its entirety, upon 90 days written notice to Pfizer. Either party has the right to terminate the license agreement in connection with an insolvency event involving the other party or a material breach of the license agreement by the other party that remains uncured for a specified period of time. If the license agreement is terminated by either party for any reason, worldwide rights to the research, development, manufacture and commercialization of the products revert back to Pfizer.

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., Celgene, 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. (a division of Dainippon Sumitomo Corp), Stemline Therapeutics, Inc. and others.

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 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 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 third-party manufacturers. We obtain our supplies from these manufacturers 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 manufacturers 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 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. 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 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 $2.3 million, and the sponsor of an approved NDA is also subject to annual product and establishment user fees, currently exceeding $110,000 per product and $560,000 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 FDA has committed to review most such applications for non-priority products within 10 months after accepting the application for filing, and most applications for priority review products, that is, drugs that the FDA determines represent a significant improvement over existing therapy, within six months after accepting the application for filing. 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 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 accepted for filing. 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 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.

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 after the NDA or patent holder's receipt of the Paragraph IV certification, 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 for the conditions of use covered by the exclusivity, 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 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 guidance that 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 guidance issued in August 2014 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 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 our product candidates. 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.

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 an approval order, which may be for more limited indications than those originally sought by the manufacturer. The approval order 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 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 patient's safety in the study. The FDA has confirmed that one of our IVDs does not need an IDE application as it does not pose significant risk at this time. Should interim clinical trial data detect a safety signal between patients who test positive or negative for the specific biomarker, then a IDE would be required.

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 regulations requiring 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.

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.

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.

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 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 February 27, 2015, we had 47 full-time employees, including a total of 12 employees with M.D. or Ph.D. degrees. Of these full-time employees, 31 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.

BUSINESS—EXECUTIVE OFFICERS OF THE REGISTRANT

The following table sets forth the name, age and position of each of our executive officers as of February 27, 2015.

Name	Age	Position
Christoph Westphal, M.D., Ph.D.	46	Executive Chairman
Robert Forrester	51	President, Chief Executive Officer
John Green	60	Chief Financial Officer
Joanna Horobin, M.B., Ch.B.	60	Chief Medical Officer
Daniel Paterson	53	Chief Operating Officer
Monica Kleinman	34	Vice President and General Counsel

Christoph Westphal, M.D., Ph.D. has served as our Executive Chairman since July 2013, as our Chief Executive Officer from September 2011 until July 2013 and our President from September 2011 until January 2013. 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 and the Chairman and CEO of Flex Pharma, Inc., since 2014. 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.

Robert Forrester has served has served as our Chief Executive Officer since July 2013, as our Chief Operating Officer from March 2011 until July 2013 and our President since January 2013. 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. from 2009 until 2010 and as its Executive Vice President and Chief Financial Officer from 2004 to 2009. Mr. Forrester 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.

John (Jack) Green has served as our Chief Financial Officer since May 2013. Prior to joining the Company, from March 2011 until November 2012, Mr. Green was Vice President, Finance and a key member of the senior management team for On-Q-ity and oversaw the financial activities, planning, budgeting and management of commercial and banking relationships. Previously, from May 2002 until June 2010, Mr. Green was the Senior Vice President and Chief Financial Officer of GTC Biotherapeutics (formerly Genzyme Transgenics Corporation) which was spun out from Genzyme Corporation as a stand-alone public company. Mr. Green is a Certified Public Accountant (CPA) with over 30 years of financial experience, including 20 within the biotechnology industry. Mr. Green received a Master's degree in Business Administration from Boston University Graduate School of Management and a Bachelor's degree from the College of the Holy Cross.

Joanna Horobin, M.B., Ch.B. has served as our Chief Medical Officer since October 2012. Dr. Horobin has 30 years of pharmaceutical drug development experience. Prior to joining us, Dr. Horobin served as President of Syndax Pharmaceuticals from April 2012 to October 2012 and President and CEO from May 2006 to April 2012. At Syndax, Dr. Horobin designed and implemented the phase 2 clinical development of Entinostat in metastatic breast cancer. Entinostat has been designated breakthrough designation by the FDA. Previously, she served as VP, Oncology at Rhone-Poulenc Rorer (now Sanofi), COO of CombinatoRx and EVP at EntreMed with experience spanning clinical development, marketing, and general management in both public and private companies. At Rhone-Poulenc Rorer (now Sanofi) Dr. Horobin led the global oncology business, including the successful global launch of Taxotere® (docetaxel) in breast cancer and Campto/Camptosar® (CPT11) for colorectal cancer. Dr. Horobin also led a successful joint venture with Chugai to launch Granocyte® (lenograstim). Previously, Dr. Horobin played significant leadership roles in the approvals of Lovenox®, Celectol®, Augmentin®, Timentin®, temocillin, Bactroban® and Relafen®/Reliflex®. Dr. Horobin received her medical degree from the University of Manchester, England. While in clinical practice she gained membership to the Royal College of General Practitioners in the United Kingdom and holds a diploma of pharmaceutical medicine from the Royal College of Physicians in the United Kingdom.

Daniel Paterson has served as our Chief Operating Officer since December 2014, our Chief Business Officer from July 2013 to December 2014 and as our Vice President, Head of Corporate

Development and Diagnostics from March 2012 until July 2013. Prior to joining us, Mr. Paterson was a consultant in 2011 until joining us in 2012. From 2009 through 2010, Mr. Paterson was the COO of On-Q-ity. Mr. Paterson was the President and CEO of The DNA Repair Company from 2006 until 2009, when it was acquired by On-Q-ity. Previously, he held senior level positions at IMS Health, CareTools, OnCare and Axion.

Monica Kleinman has served as our General Counsel since March 2014. Prior to joining the Company, Ms. Kleinman was a member of the securities and public company group at Ropes & Gray LLP where she advised on corporate transactions involving public companies, investment banks and private equity funds and provided counsel on securities law compliance and corporate governance matters. Ms. Kleinman holds a B.A. from Amherst College and a J.D. from Harvard Law School.

OUR CORPORATE INFORMATION

We were incorporated under the laws of the State of Delaware in August 2010. Our principal executive offices are located at 117 Kendrick Street, Suite 500, Needham, Massachusetts 02494 and our telephone number is (781) 292- 4200.

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.

ITEM 1A. Risk Factors.

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

Preclinical studies and preliminary and interim data from clinical trials of our product candidates are not necessarily predictive of the results or success of ongoing or later clinical trials of our product candidates. If we cannot replicate the results from our preclinical studies and clinical trials of our product candidates, we may be unable to successfully develop, obtain regulatory approval for and commercialize our product candidates.

Preclinical studies and any positive preliminary and interim data from our clinical trials of our product candidates may not necessarily be predictive of the results of ongoing or later clinical trials. Even if we are able to complete our planned clinical trials of our product candidates according to our current development timeline, the positive results from clinical trials of our product candidates may not be replicated in subsequent clinical trial results. Also, our later-stage clinical trials, including our registration-directed study of VS-6063 in mesothelioma, could differ in significant ways from our ongoing Phase 1 and Phase 2 clinical trials of VS-6063, which could cause the outcome of these later-stage trials to differ from our earlier stage clinical trials. For example, these differences may include changes to inclusion and exclusion criteria, efficacy endpoints and statistical design. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials after achieving positive results in early-stage development, and we cannot be certain that we will not face similar setbacks. We have not completed any late-stage clinical trials for our product candidates yet, and if we fail to produce positive results in our planned clinical trials of any of our product candidates, the development timeline and regulatory approval and commercialization prospects for our product candidates, and, correspondingly, our business and financial prospects, would be materially adversely affected.

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 research and development of our lead product candidate, VS-6063. We are conducting clinical trials of VS-6063 in multiple indications, including a registration-directed trial in mesothelioma. Our other product candidates, VS-5584 and VS-4718, are in Phase 1 clinical trials. Our ability to generate product revenues, which we do not expect will occur for several years, if ever, will depend heavily on the successful development and eventual commercialization of our product candidates, particularly VS-6063. The success of our product candidates will depend on several factors, including the following:

- successful completion of these clinical trials, particularly the registration-directed trial of VS-6063 in mesothelioma;
- receipt of marketing approvals from the FDA and other regulatory authorities for our product candidates, particularly VS-6063 in mesothelioma, including pricing approvals where required;
- 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;
- establishing commercial capabilities, including hiring and training a sales force, and 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 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. For example, the results of many of our ongoing clinical trials to date, including our Phase 2 window-of-opportunity study of VS-6063 in mesothelioma and our Phase 1/1b study of VS-6063 in ovarian cancer, are based on unaudited data provided by our clinical trial investigators. An audit of this data may change the conclusions drawn from this unaudited data indicating less promising results than we currently anticipate. In addition, our registration-directed clinical trial of VS-6063 in mesothelioma is a randomized, double-blind, placebo-controlled study. Accordingly, while we will know whether the trial can proceed and the primary patient population at the time of the interim analysis expected in the second quarter of 2015, we will not have any safety or activity data from this trial until the primary efficacy readout in 2016.

In some instances, there can be significant variability in safety and/or efficacy results between different trials of the same product candidate due to numerous factors, including changes in trial protocols, differences in size and type of the patient populations, adherence to the dosing regimen and other trial protocols and the rate of dropout among clinical trial participants. We do not know whether any Phase 2, Phase 3 or other clinical trial we may conduct will demonstrate consistent or adequate efficacy and safety sufficient to obtain regulatory approval to market our product candidates.

In addition, the design of a clinical trial may determine whether its results will support approval of a product and flaws in the design of a clinical trial may not become apparent until the clinical trial is well-advanced. 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 clinical trial contracts or clinical trial protocols with prospective trial sites;
- clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development programs;
- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- regulators or institutional review boards may require that we or our investigators suspend or terminate clinical trials 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.

We are currently conducting a registration-directed trial of VS-6063 in mesothelioma. The key endpoints for this trial are progression free survival, meaning the length of time on treatment until objective tumor progression, overall survival and quality of life. We have no clinical data in humans relating to the impact of VS-6063 on overall survival or progression free survival. If VS-6063 does not demonstrate a progression free or overall survival benefit or does not meet the level of statistical or clinical significance required by the FDA and other regulatory authorities for marketing approval, it will not be approved. We have met with regulatory agencies in the US, UK and Japan and interacted with regulatory agencies in eleven other countries. Based on these interactions, we believe that positive results from this trial, if obtained, may enable us to seek regulatory approval for VS-6063. However, the FDA and foreign regulatory authorities may determine that the results from this trial do not support regulatory approval and may require us to conduct an additional clinical trial or trials. If these agencies take such a position, the costs of development of VS-6063 in mesothelioma could increase materially and its potential market introduction could be delayed. The regulatory agencies could also require that we conduct additional clinical, nonclinical or manufacturing validation studies and submit

that data before it will consider an NDA application. Our product development costs will also increase if we experience delays in clinical 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, there are a number of ongoing clinical trials for product candidates treating cancer. Patients who would otherwise be eligible for our clinical trials may instead enroll in clinical trials of our competitors' product candidates, particularly if they view such treatments to be more conventional and established.

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.

For example, we may have difficulty enrolling a sufficient number of patients in our registration-directed trial of VS-6063 in mesothelioma due to the relatively small patient population and the rapid progression of the disease. If we are unable to enroll patients in this trial as quickly as we planned, the interim analysis currently anticipated for the second quarter of 2015 could be delayed, which would also delay the expected timing of the primary efficacy read-out and any potential NDA filing.

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 unexpected 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 in various stages of clinical 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. For example, even though VS-6063 has generally been well-tolerated by patients in our clinical trials to date, there were side effects or adverse events, some of which were serious. The most common drug-related adverse events, or AEs, from our open-label single-agent clinical trials with VS-6063 alone were nausea,

fatigue, increased serum bilirubin, diarrhea, dyspnea, vomiting, decreased appetite, constipation, cough and headache. These AEs were mild, moderate or severe in severity. The most common AEs in open-label single-agent clinical trials assessed as Grade 3 or severe were constipation, dyspnea, fatigue, headache, increased serum bilirubin, nausea and vomiting. The most common drug-related AEs in the combination study with VS-6063 and paclitaxel were fatigue, anemia, hyperbilirubinemia, diarrhea, nausea, neutropenia, peripheral edema, vomiting, pyrexia, alopecia, decreased appetite, dizziness, peripheral sensory neuropathy, urinary tract infection, upper abdominal pain, dehydration and peripheral neuropathy. These AEs were mild, moderate, or severe in intensity. The most common AEs in the combination study assessed as Grade 3 (severe) were anemia, dehydration, fatigue, hyperbilirubinemia, nausea, neutropenia, pyrexia, urinary tract infection and vomiting. A small number of patients across our clinical trials have experienced serious adverse events, or SAEs, deemed by us and the clinical investigator to be related to VS-6063. SAEs refer to AEs that result in death, are life threatening, require hospitalization or prolonging of hospitalization, or cause a significant and permanent disruption of normal life functions, congenital anomalies or birth defects, or require intervention to prevent such an outcome. Reports of SAEs from our open-label Phase 1 and Phase 2 trials of VS-6063 deemed by us and the clinical investigator to be related to VS-6063 included dehydration, dyspnea, nausea, renal failure, respiratory failure and vomiting.

VS-5584 has also been generally well tolerated in our Phase 1 clinical trial, and the toxicities were consistent with other drugs in this class. The most frequent AEs were nausea, fatigue, constipation, diarrhea, hyperglycemia, decreased appetite, mucosal inflammation, vomiting, anemia, back pain, dizziness, hypertriglyceridemia, hypokalemia, and hyponatremia. Toxicities were generally Grade 1 or 2 in severity. There has been one treatment-related unexpected SAE reported to date, a report of a Grade 3 rash.

VS-4718 has been generally well tolerated in our Phase 1 clinical trial. The most frequently reported AEs include nausea, fatigue, dyspnea, agitation, diarrhea, and maculo-papular rash. One treatment-related SAE of increased aspartate aminotransferase (AST) and alanine aminotransferase (ALT) (Grade 3) was reported.

As a result of these adverse events or further safety or toxicity issues that we may experience in our clinical trials in the future, we may not receive approval to market any product candidates, which could prevent us from ever generating revenue from the sale of products or achieving profitability. Results of our trials could reveal an unacceptably high severity and prevalence of side effects. In such an event, our trials could be suspended or terminated and the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny approval of our products candidates for any or all targeted indications. 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. In addition, while we and our clinical trial investigators, currently determine if serious adverse or unacceptable side effects are drug-related, the FDA or other non-U.S. regulatory authorities may disagree with our or our clinical trial investigators' interpretation of data from clinical trials and the conclusion that a serious adverse effect or unacceptable side effect was not drug-related.

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.

We are discovering and developing product candidates to treat cancer by the targeted killing of cancer stem cells. Research on CSCs is an emerging field and, consequently, there is ongoing debate regarding the existence of CSCs, whether the appropriate nomenclature to refer to these cells is cancer stem cells, tumor-initiating cells or another term and the importance of these cells as an underlying cause of tumor recurrence and metastasis.

Although there is general consensus that some cancer cells have tumor- initiating capacity, there also is some debate in the scientific community regarding the defining characteristics of these cells,

which we call CSCs, and the origin of these cells. Some believe that normal adult stem cells mutate and transform into CSCs. Others believe that all cancer cells have tumor-initiating capabilities, these capabilities cannot be attributed to a factor intrinsic to a particular cell and, therefore, a definitive CSC cannot be isolated or targeted. We believe that the discovery by our scientific co-founders of the link between the epithelial-to-mesenchymal transition, or EMT, and the emergence of cancer stem cells is one way a cancer cell can transition to a CSC, but this view is not universally accepted. In addition, some believe that targeting CSCs should be sufficient for a positive clinical outcome, while others believe that, at times or always, targeting CSCs should be coupled with targeting tumor bulk for a positive clinical outcome.

Even if our beliefs regarding the existence, characteristics and function of CSCs are correct, any products 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. While we are currently conducting clinical trials for product candidates that we believe target CSCs, we may not ultimately be successful in demonstrating their efficacy, alone or in combination with other treatments.

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

Part of our strategy involves discovering and developing product candidates, including through our proprietary technology, to build a pipeline of novel product candidates. 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;
- 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; or
- potential product candidates may not be effective in treating their targeted diseases.

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

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

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

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

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.

If we fail to obtain regulatory approval in jurisdictions outside the United States, we will not be able to market our products in those jurisdictions.

We intend to seek regulatory approval for our product candidates in a number of countries outside of the United States and expect that these countries will be important markets for our products, if approved. For example, our registration-directed study of VS-6063 in mesothelioma is currently in twelve countries outside of the United States, including the United Kingdom, Japan, Australia and South Africa. Marketing our products in these countries will require separate regulatory approvals in each market and compliance with numerous and varying regulatory requirements. The regulations that apply to the conduct of clinical trials and approval procedures vary from country to country and may require additional testing. Moreover, the time required to obtain approval may differ from that required to obtain FDA approval. In addition, in many countries outside the United States, drugs must be approved for reimbursement before it can be approved for sale in that country. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. The foreign regulatory approval process may include all of the risks associated with obtaining FDA approval. We may not obtain foreign regulatory approvals on a timely basis, if at all. We may not be able to file for regulatory approvals and may not receive necessary approvals to commercialize our products in any foreign market.

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. In addition, if we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

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

If, at the interim analysis of our registration-directed trial of VS-6063 in mesothelioma, we enrich the study population based on the biomarker merlin, we plan to develop a companion diagnostic. 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 limited experience in the development of diagnostics and may not be successful in developing appropriate diagnostics to pair with our product candidates, including VS-6063. 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. We have entered into agreements with the Laboratory Corporation of America Holdings to assist us in validating biomarkers for VS-6063, and establishing, validating and performing a test for merlin protein being used for stratification in the COMMAND study, and in the development of an applicable companion diagnostic, if necessary. If we or any third parties that we engage to assist us, are unable to successfully develop companion diagnostics for our product candidates, or experience delays in doing so:

- the development of our product candidates may be adversely affected if we are unable to appropriately select patients for enrollment in our clinical trials;
- our product candidates may not receive marketing approval if safe and effective use of a product candidate depends on an in vitro diagnostic; and
- we may not realize the full commercial potential of any product candidates 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 instead of trying new single-agent therapies. If our product candidates do not achieve an adequate level of acceptance, we may not generate significant product revenues and we may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

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

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

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

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

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

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

In addition, given the relatively small patient population for mesothelioma in the United States and the other geographies where we intend to commercialize VS-6063, assuming regulatory approval of VS-6063 in mesothelioma, additional sales personnel may be required to access the same number of patients as it would for a more common cancer.

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

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

The development and commercialization of new drug products is highly competitive. We face competition with respect to our current product candidates, and will face competition with respect to any product candidates that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of the disease indications for which we are developing our product candidates. Some of these competitive products and therapies are based on scientific approaches that are the same as or similar to our approach, and

others are based on entirely different approaches. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

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

There are also a number of products in clinical development by third parties to treat cancer by targeting CSCs. These companies include divisions of large pharmaceutical companies, including Astellas Pharma US, Inc., Sanofi-Aventis US LLC, GlaxoSmithKline plc, Boehringer Ingelheim GmbH, Pfizer Inc, Celgene, Inc. and others. There are also biotechnology companies of various sizes that are developing therapies against CSCs, including OncoMed Pharmaceuticals, Inc., Boston Biomedical, Inc. (a division of Dainippon Sumitomo Corp.), Stemline Therapeutics, Inc. and others. 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.

In addition, to the extent that product or product candidates of our competitors demonstrate serious adverse side effects or are determined to be ineffective in clinical trials, the development of our product candidates could be negatively impacted.

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

The regulations that govern marketing approvals, pricing and reimbursement for new drug products vary widely from country to country. In the United States, recently passed legislation may significantly change the purchase of pharmaceutical products, resulting in lower prices and a reduction in product demand. 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.

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 $10.0 million in product liability insurance coverage in the aggregate, with a per incident limit of $10.0 million, which may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage as we initiate additional clinical trials in the United States and around the world or upon 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.

RISKS RELATED TO OUR FINANCIAL POSITION AND NEED FOR ADDITIONAL CAPITAL

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

Since inception, we have incurred significant operating losses. As of December 31, 2014, we had an accumulated deficit of $141.0 million. To date, we have not generated any revenues and have financed our operations through private placements of our preferred stock, public offerings of our common stock and sales of our common stock pursuant to our at-the-market equity offering program. We have devoted substantially all of our efforts to research and development. Our lead product candidate, VS-6063, is currently in a registration-directed trial in mesothelioma (COMMAND). Our other product candidates, VS-5584 and VS-4718, and VS-6063 in indications outside of mesothelioma are in Phase 1 and Phase 2 clinical trials. We expect that it will be several 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 COMMAND study, including the initiation of associated studies in preparation for a possible NDA filing to the FDA and similar filings to other regulatory authorities;

- continue our other ongoing clinical trials with VS-6063, VS-5584 and VS-4718;
- initiate additional clinical trials for our product candidates;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- continue our preclinical research or acquire or in-license additional product candidates;
- maintain, expand and protect our intellectual property portfolio;
- hire additional clinical, development 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. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of the company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

We will continue to 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 clinical development of our product candidates, including preparation for a potential NDA filing for VS-6063 in mesothelioma. In addition, if we obtain marketing approval for any of our product candidates, including VS-6063 in mesothelioma, 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 clinical development programs or any future commercialization efforts.

We expect our existing cash, cash equivalents and investments, including $55.5 million of net proceeds received in January 2015 from the sale of our common stock, will enable us to fund our current operating plan and capital expenditure requirements into 2017. Our future capital requirements will depend on many factors, including:

- the rate of enrollment, results and cost of completing the registration-directed trial of VS-6063 in mesothelioma;
- assuming favorable clinical results, the cost, timing and outcome of our efforts to seek approval in mesothelioma in the United States and elsewhere in the world, including to fund the preparation and filing of regulatory submissions with the FDA and other regulatory agencies worldwide;
- assuming regulatory approval, the costs of future commercialization activities, including product sales, marketing, manufacturing and distribution, of VS-6063 in mesothelioma in the United States and elsewhere in the world, whether alone or through a third party;

- the scope, progress and, results of our other ongoing clinical trials and potential future clinical trials;
- the extent to which we acquire or in-license other product candidates;
- the costs, timing and outcome of regulatory review of our other product candidates and the costs of future commercialization activities for such 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 or partnerships on favorable terms, if at all.

Conducting 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 may not be commercially available for several years, if at all. Moreover, our registration-directed trial is in mesothelioma, which has a relatively low incidence as compared to other cancer indications. Accordingly, even if we receive regulatory approval following this trial, it will take several years to achieve peak sales and we will need to continue to rely on additional financing to further our clinical development 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 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, grants and government funding, 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, 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 future revenue streams or valuable rights to 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 OUR DEPENDENCE ON THIRD PARTIES

We may 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. We anticipate that we may seek to enter into a collaboration for marketing and

commercialization of VS-6063 in certain territories worldwide at the appropriate time in the future. 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 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.

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. 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 certain product candidates, reduce or delay our development programs, delay 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 rely in part on third parties to conduct our clinical trials and preclinical testing, and if they do not properly and successfully perform their obligations to us, we may not be able to obtain regulatory approvals for our product candidates.

We rely on third parties, such as contract research organizations, clinical data management organizations, medical institutions and clinical investigators, to conduct, provide monitors for and manage data from our clinical trials. For example, we currently rely on third party CROs to conduct our registration-directed trial of VS-6063 in mesothelioma, our Phase 1b trial of VS-6063 in combination with weekly paclitaxel for patients with ovarian cancer, our Phase 2 study of VS-6063 in patients with non-small cell lung cancer, our Phase 1 trial of VS-6063 and VS-5584 in patients with mesothelioma and our Phase 1 trials of VS-4718 and VS-5584 in patients with advanced cancers. We compete with many other companies for the resources of these third parties. 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 and ultimately the commercialization of our product candidates.

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, or GCP, 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. Regulatory authorities enforce these GCP requirements through periodic inspections of trial sponsors, principal investigators and trial sites. If we or any of our CROs fail to comply with applicable GCP requirements, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or other regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials comply with GCP requirements. 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.

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 intend to rely on third parties to conduct investigator-sponsored clinical trials of VS-6063 and our other product candidates. Any failure by a third party to meet its obligations with respect to the clinical development of our product candidates may delay or impair our ability to obtain regulatory approval for VS-6063 and our other product candidates.

We intend to rely on academic and private non-academic institutions to conduct and sponsor clinical trials relating to VS-6063 and our other product candidates. We will not control the design or conduct of the investigator-sponsored trials, and it is possible that the FDA or non-U.S. regulatory authorities will not view these investigator-sponsored trials as providing adequate support for future clinical trials, whether controlled by us or third parties, for any one or more reasons, including elements of the design or execution of the trials or safety concerns or other trial results.

Such arrangements will provide us certain information rights with respect to the investigator-sponsored trials, including access to and the ability to use and reference the data, including for our own regulatory filings, resulting from the investigator-sponsored trials. However, we do not have control over the timing and reporting of the data from investigator-sponsored trials, nor do we own the data from the investigator-sponsored trials. If we are unable to confirm or replicate the results from the investigator-sponsored trials or if negative results are obtained, we would likely be further delayed or prevented from advancing further clinical development of our product candidates. Further, if investigators or institutions breach their obligations with respect to the clinical development of our product candidates, or if the data proves to be inadequate compared to the first-hand knowledge we might have gained had the investigator-sponsored trials been sponsored and conducted by us, then our ability to design and conduct any future clinical trials ourselves may be adversely affected.

Additionally, the FDA or non-U.S. regulatory authorities may disagree with the sufficiency of our right of reference to the preclinical, manufacturing or clinical data generated by these investigator-sponsored trials, or our interpretation of preclinical, manufacturing or clinical data from these investigator-sponsored trials. If so, the FDA or other non-U.S. regulatory authorities may require us to obtain and submit additional preclinical, manufacturing, or clinical data before we may initiate our planned trials and/or may not accept such additional data as adequate to initiate our planned trials.

We contract with third parties for the manufacture of our product candidates and for compound formulation research, and these third parties may not perform satisfactorily.

We do not have any manufacturing facilities or personnel. We currently obtain all of our supply of our product candidates for clinical development from third-party manufacturers or third-party collaborators, and we expect to continue to rely on third parties for the manufacture of clinical and, if necessary, commercial quantities of our product candidates. In addition, we currently rely on third parties for the development of various formulations of our product candidates. We obtain our supplies from these manufacturers on a purchase order basis, and we do not have any long term supply agreements in place. This reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or such quantities at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts.

Any of these third parties may terminate their engagement with us at any time. We do not currently have arrangements in place for redundant supply or a second source for bulk drug substance.

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, including the misappropriation of our proprietary information, trade secrets and know-how;
- the possible termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us; and
- disruptions to the operations of our manufacturers or suppliers caused by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier or a catastrophic event affecting our manufacturers or suppliers.

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.

If our current contract manufacturers 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, as well as producing the drug product. In addition, we have to enter into technical transfer agreements and share our know-how with the third-party manufacturers, which can be time-consuming and may result in delays.

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, The Scripps Research Institute, or Scripps, and Pfizer, Inc., or Pfizer, and expect to enter into additional license agreements in the future. In particular, we have exclusively licensed rights to our lead product candidate, VS-6063, from Pfizer. 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, Scripps, and Pfizer, 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

Scripps were to terminate its license agreement with us for any reason, we would lose our rights to VS- 4718. If Pfizer were to terminate its license agreement with us for any reason, we would lose our rights to VS-6063, which is our lead product candidate, and currently being used in five ongoing clinical trials.

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

Our success depends in large part on our and our licensors' ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary technology and products. We and our licensors seek to protect our proprietary position by filing patent applications in the United States and abroad related to our novel technologies and products that are important to our business. We cannot be certain that any patents will issue with claims that cover our proprietary 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 Scripps. 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, in the United States, for patents that have an effective filing date prior to March 15, 2013, 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 transitioned to a first inventor to file system in which, assuming the other requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent. We may be subject to a third party preissuance submission of prior art to the U.S. Patent and Trademark Office, or become involved in opposition, derivation, reexamination, inter parties review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights.

Even if our owned and licensed patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner.

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

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

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

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

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing the proprietary rights of third parties. We have yet to conduct comprehensive freedom-to-operate searches to determine whether our use of certain of the patent rights owned by or licensed to us would infringe patents issued to third parties. We may become party to, or threatened

with, future adversarial proceedings or litigation regarding intellectual property rights with respect to our products and technology, including interference proceedings before the U.S. Patent and Trademark Office. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future. If we are found to infringe a third party's intellectual property rights, we could be required to obtain a license from such third party to continue developing and marketing our products and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. We could be forced, including by court order, to cease commercializing the infringing technology or product. In addition, we could be found liable for monetary damages. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

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

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

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

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

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

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

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

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

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

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

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

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

We have received orphan disease status for certain of our product candidates, but there can be no assurance that we will be able to prevent third parties from developing and commercializing products that are competitive to these product candidates.

We received orphan drug designation in the U.S. and the EU for the use of VS-6063 and VS-5584 in mesothelioma. If either of these product candidates obtains marketing authorization, it will receive orphan drug exclusivity. Orphan drug exclusivity grants seven years of marketing exclusivity under the Federal Food, Drug and Cosmetic Act and up to 10 years of marketing exclusivity in Europe. A competitor may receive orphan drug marketing authorization prior to us for the same indication for which we are developing these product candidates. Other companies have received orphan drug designations for compounds other than VS-6063 and VS-5584 for mesothelioma in the U.S. and EU. While orphan drug exclusivity for VS-6063 or VS-5584 would provide market exclusivity, we would not be able to exclude other companies from manufacturing and/or selling drugs using the same active ingredient for the same indication beyond that timeframe on the basis of orphan drug designation. Furthermore, the marketing exclusivity in Europe can be reduced from 10 years to six years if the initial designation criteria have significantly changed since the market authorization of the orphan medicinal product. We cannot guarantee that another company also with orphan drug designation will not receive marketing authorization for the same indication before we do. If that were to happen, our applications for that indication may not be approved until the competing company's period of exclusivity has expired. Even if we are the first to obtain marketing authorization for an orphan drug indication, there are circumstances under which a competing product may be approved for the same indication during the seven-year period of marketing exclusivity, such as if the later product is shown to be clinically superior to our product, or if the later product is a different drug than our product candidate. Further, the seven-year marketing exclusivity would not prevent competitors from obtaining approval of the same compound for other indications or the use of other types of drugs for the same use as the orphan drug.

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

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

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

- restrictions on such products, manufacturers or manufacturing processes;

- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing clinical trials;
- warning or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenue;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our products;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

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

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

- the federal healthcare anti-kickback statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs such as Medicare and Medicaid;
- the federal False Claims Act imposes criminal and civil penalties, including civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;
- the federal Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Economic and Clinical Health Act, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program and also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the federal false statements statute prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services;

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

- analogous state laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, and some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other health care providers or marketing expenditures.

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.

Our employees, independent contractors, principal investigators, CROs, consultants and vendors may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements, which could cause significant liability for us and harm our reputation.

We are exposed to the risk that our employees, independent contractors, principal investigators, CROs, consultants and vendors may engage in fraud or other misconduct, including intentional failures to: comply with FDA regulations or similar regulations of comparable foreign regulatory authorities, provide accurate information to the FDA or comparable foreign regulatory authorities, comply with manufacturing standards we have established, comply with federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by comparable foreign regulatory authorities, report financial information or data accurately or disclose unauthorized activities to us. Such misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter misconduct by employees and other third parties, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws, standards or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of significant fines or other sanctions.

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

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

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

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 chief executive officer and other key executives and to attract, retain and motivate qualified personnel.

We are highly dependent on Robert Forrester, our President and Chief Executive Officer, Joanna Horobin, our Chief Medical Officer and Daniel Paterson, our Chief Operating Officer, as well as the other principal members of our management and scientific teams. Although we have formal employment agreements with Robert Forrester, Joanna Horobin and Daniel Paterson, these agreements do not prevent them from terminating their employment with us at any time. 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.

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 have experienced and expect to continue 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.

Our business and operations may be materially adversely affected in the event of computer system failures.

Despite the implementation of security measures, our internal computer systems, and those of our contract research organizations and other third parties on which we rely, are vulnerable to damage from computer viruses, unauthorized access, natural disasters, fire, terrorism, war and telecommunication and electrical failures. If such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our clinical development programs. For example, the loss of clinical trial data from ongoing or planned clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development of our product candidates could be delayed.

RISKS RELATED TO OUR COMMON STOCK

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

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

- establish a classified board of directors such that not all members of the board are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;

- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a "poison pill" that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws.

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

The market price of our common stock has been, and may continue to be, highly volatile.

Our stock price has been volatile. Since January 27, 2012, when we became a public company, the price for one share of our common stock has reached a high of $18.82 and a low of $6.25. We cannot predict whether the price of our common stock will rise or fall. The market price for our common stock may be influenced by many factors, including:

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

In addition, the stock market in general and the market for small pharmaceutical companies and biotechnology companies in particular have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of particular companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. In the past, following periods of volatility in the market, securities class-action litigation has often been instituted against companies. Such litigation, if instituted against us, could result in substantial costs and diversion of management's attention and resources, which could materially and adversely affect our business and financial condition.

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.

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

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

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

We cannot predict whether investors will find our common stock less attractive because we will rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

Item 1B. Unresolved Staff Comments

None.

Item 2. Properties

We occupy approximately 15,197 square feet of office and laboratory space in Needham, Massachusetts under a lease that expires in September 2019. 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

Not applicable.

PART II

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

MARKET INFORMATION

Our common stock is publicly traded on the NASDAQ Global Market under the symbol "VSTM." The following table sets forth, for the periods indicated, the high and low sales prices for our common stock as reported on the NASDAQ Global Market.

Year ended December 31, 2014	High	Low
First quarter	$ 16.60	$ 10.29
Second quarter	$ 11.47	$ 7.10
Third quarter	$ 10.06	$ 7.28
Fourth quarter	$ 10.31	$ 8.01

Year ended December 31, 2013	High	Low
First quarter	$ 11.75	$ 8.78
Second quarter	$ 14.09	$ 8.27
Third quarter	$ 18.82	$ 12.38
Fourth quarter	$ 13.47	$ 8.60

HOLDERS

As of February 27, 2015, there were 38 holders of record of our common stock and the closing price of our common stock on the NASDAQ Global Market as of that date was $7.44. The number of holders of record 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, 2014. There are no equity compensation plans that have not been approved by our security holders.

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
Equity compensation plans approved by security holders(1)	4,206,440	$ 10.38	575,086(3)
Equity compensation plans not approved by security holders(2)	—	—	750,000

(1) Includes information regarding our 2010 Equity Incentive Plan and 2012 Incentive Plan.

(2) The Board of Directors has authorized and reserved 750,000 shares of common stock that may be issued pursuant to stock options granted or to be granted to new employees in accordance with NASDAQ Listing Rule 5635(c)(4), as an inducement material to such employees entering into employment with the Company. The terms of these stock options are consistent with stock options granted under the Company's 2012 Incentive Plan.

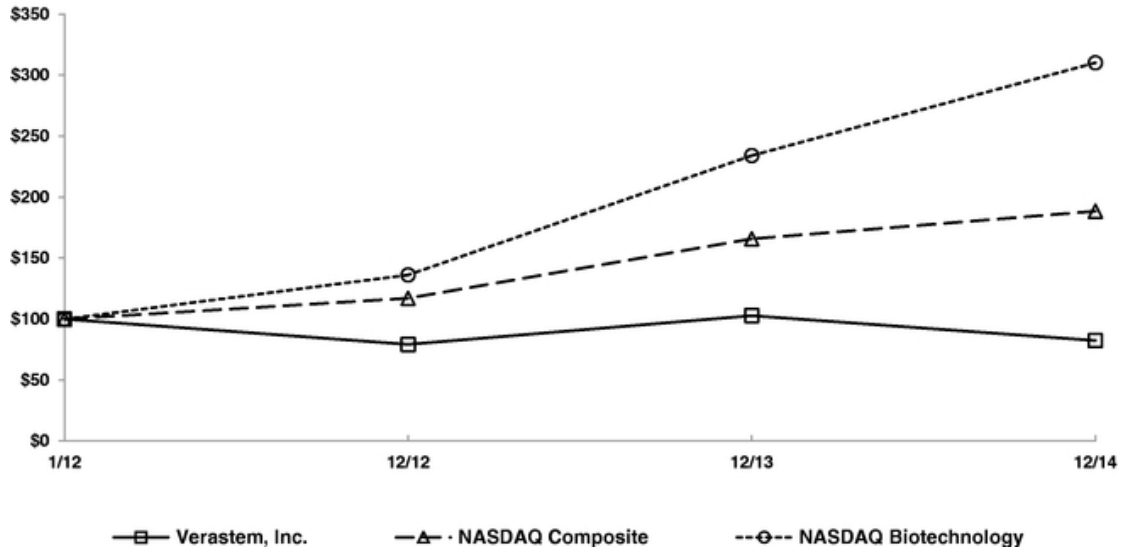
(3) Does not include 1,081,045 shares added to the 2012 Incentive Plan under the evergreen provision on January 1, 2015.

PERFORMANCE GRAPH

The following performance graph and related information shall not be deemed to be "soliciting material" or to be "filed" with the SEC, nor shall such information be incorporated by reference into any future filing under the Securities Act, except to the extent that we specifically incorporate it by reference into such filing.

The following graph compares the performance of our common stock to the NASDAQ Composite Index and to the NASDAQ Biotechnology Index from January 27, 2012 (the first date that shares of our common stock were publicly traded) through December 31, 2014. The comparison assumes $100 was invested after the market closed on January 27, 2012 in our common stock and in each of the foregoing indices, and it assumes reinvestment of dividends, if any.

COMPARISON OF 3 YEAR CUMULATIVE TOTAL RETURN*
Among Verastem, Inc., the NASDAQ Composite Index, and the NASDAQ Biotechnology Index



* $100 invested on 1/27/12 in stock or 12/31/11 in index, including reinvestment of dividends. Fiscal year ending December 31, 2014.

Cumulative Total Return Comparison

	1/27/12	12/31/12	12/31/13	12/31/14
Verastem, Inc.	**100.00**	**79.26**	**102.80**	**82.42**
NASDAQ Composite	**100.00**	**117.11**	**165.83**	**188.50**
NASDAQ Biotechnology	**100.00**	**136.21**	**234.11**	**310.21**

PURCHASE OF EQUITY SECURITIES

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

Item 6. Selected Financial Data

You should read the following selected financial data together with our consolidated 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. The selected historical financial information in this section is not intended to replace our financial statements and the related notes therein. Our historical results for any prior period are not necessarily indicative of results to be expected in any future period.

Statement of operations data:	Period from August 4, 2010 (inception) to December 31, 2010	Years Ended December 31, 2011	2012	2013	2014
		(in thousands, except share and per share amounts)			
Operating expenses:					
Research and development	$ 400	$ 9,883	$ 21,712	$ 25,930	$ 35,448
General and administrative	384	3,815	10,518	15,472	18,159
Total operating expenses	784	13,698	32,230	41,402	53,607
Loss from operations	(784)	(13,698)	(32,230)	(41,402)	(53,607)
Interest income	—	15	246	200	242
Net loss	(784)	(13,683)	(31,984)	(41,202)	(53,365)
Accretion of preferred stock	(2)	(32)	(6)	—	—
Net loss applicable to common stockholders	$ (786)	$ (13,715)	$ (31,990)	$ (41,202)	$ (53,365)
Net loss per share applicable to common stockholders—basic and diluted	$ (0.91)	$ (10.59)	$ (1.70)	$ (1.82)	$ (2.07)
Weighted-average number of common shares used in net loss per share applicable to common stockholders—basic and diluted	850	1,295	18,765	22,680	25,804

Balance sheet data:	As of December 31, 2010	2011	2012	2013	2014
			(in thousands)		
Cash, cash equivalents and investments	$ 3,584	$ 56,805	$ 91,520	$ 123,656	$ 92,675
Working capital	3,228	44,795	54,683	94,151	86,112
Total assets	3,604	59,037	92,923	125,261	98,649
Redeemable convertible preferred stock	3,923	68,141	—	—	—
Accumulated deficit	(784)	(14,467)	(46,451)	(87,653)	(141,018)
Total stockholders' (deficit) equity	(687)	(12,766)	90,466	117,446	88,766

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 consolidated financial statements and related notes appearing elsewhere in this Annual Report on Form 10-K. The following discussion contains forward-looking statements that involve risks and uncertainties. Our actual results and the timing of certain events could differ materially from those anticipated in these forward-looking statements as a result of certain factors, including those discussed below and as set forth under "Risk Factors." Please also refer to the section under heading "Forward-Looking Statement."

OVERVIEW

We are a biopharmaceutical company focused on discovering and developing drugs to treat cancer by the targeted killing of cancer stem cells. 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 tumors, their recurrence and metastasis. Our most advanced programs target the Focal Adhesion Kinase, or FAK, and the PI3K/mTOR signaling pathways. Our lead FAK inhibitor, VS-6063, has been assigned defactinib as the United States Adopted Name (USAN). We have received orphan drug designation for the use of VS-6063 in mesothelioma in the European Union and in the United States. VS-6063 is currently in a registration-directed trial (COMMAND) in patients with malignant pleural mesothelioma, a Phase 1b trial in combination with weekly paclitaxel for patients with ovarian cancer, a Phase 2 study in patients with non-small cell lung cancer a Phase 2 trial preceding surgery in mesothelioma and a combination trial of VS-6063 and VS-5584 in patients with relapsed mesothelioma. We expect to conduct an interim analysis of COMMAND during the second quarter of 2015 and following this and assuming the trial is not futile, we intend to communicate the specific patient population for the primary analysis. We also expect to update or announce results from the combination trial of VS-6063 and paclitaxel in patients with ovarian cancer and results from the Phase 2 study of VS-6063 in patients with non-small cell lung cancer in the second half of 2015 and to report preliminary data on the extended treatment cohort for the Phase 2 trial preceding surgery in mesothelioma in the first half of 2016. In addition to VS-6063, both our FAK inhibitor VS-4718 and our dual mTORC1/2 and PI3K inhibitor VS-5584 are in Phase 1 clinical trials in patients with advanced cancers. We have received orphan drug designation for the use of VS-5584 in mesothelioma in the European Union and in the United States. We also expect to report results from the Phase 1 trials of VS-4718 and VS-5584 in the second half of 2015.

Our operations to date have been organizing and staffing our company, business planning, raising capital, acquiring and developing our technology, identifying potential product candidates and undertaking preclinical studies and clinical trials for our 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, our initial public offering in February 2012, our follow-on offerings in July 2013 and January 2015 and sales of our common stock under our at-the-market equity offering program.

As of December 31, 2014, we had an accumulated deficit of $141.0 million. Our net loss was $53.4 million, $41.2 million and $32.0 million for the years ended December 31, 2014, 2013 and 2012, respectively. 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 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. 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.

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 several 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 product candidates. 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, clinical sites, 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. In 2014, we began to allocate the expenses related to external research and development services, such as CROs, clinical sites, manufacturing organizations and consultants by project. The table below summarizes our external allocation of research and development expenses to our clinical programs for VS-6063, VS-4718 and VS-5584, for the year ended December 31, 2014. Prior to 2014, we did not track research and development expenses for specific clinical programs. Our project costing methodology does not allocate personnel and other indirect costs to specific clinical programs. These unallocated research and development expenses are summarized in the table below and include $5.9 million of personnel costs.

	Year ended December 31, 2014 (in thousands)
VS-6063	$ 16,186
VS-4718	2,921
VS-5584	2,679
Unallocated research and development expense	9,935
Unallocated stock-based compensation expense	3,727
Total research and development expense	$ 35,448

Due to the uncertainty in drug development and the stage of development of our product candidates, we are unable to predict the requirements, specific timing and estimated costs to complete the development of our product candidates or the timing of when material cash inflows may commence, if ever.

We anticipate that our research and development expenses will increase significantly in future periods as we continue costlier development activities, including larger and later-stage clinical trials for our product candidates.

The successful development of our product candidates is highly uncertain. At 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:

- clinical trial results;
- the scope, rate of progress and expense of our research and development activities, including preclinical research and clinical trials;
- 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 receive regulatory approval for;
- 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, insurance premiums, and accounting services.

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 consolidated financial statements, which we have prepared in accordance with U.S. generally accepted accounting principles. The preparation of these consolidated 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 consolidated 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 consolidated 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 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 contract research organizations, or 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. To date, there have been no material differences between our estimates of such expenses and the amounts actually incurred.

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 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. Each of our common stock valuations 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 complete 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.

We recognize stock-based compensation expense for stock options issued to employees based on the grant date fair value of the awards on a straight-line basis over the requisite service period. We record stock-based compensation expense for stock options issued to non-employees based on the estimated fair value of the services received or of the equity instruments issued, whichever is more reliably measured, based on the vesting date fair value of the awards on a straight-line basis over the vesting period.

We estimate the fair value of stock option awards using the Black-Scholes option-pricing model. Determining the fair value of share-based awards requires the use of subjective assumptions, including the expected term of the award and expected stock price volatility. The assumptions used in determining the fair value of share-based awards represent management's best estimates, which involve inherent uncertainties and the application of management judgment. As a result, if factors change, and we use different assumptions, our share-based compensation could be materially different in the future. The risk-free interest rate used for each grant is based on a U.S. Treasury instrument whose term is consistent with the expected term of the stock option. Because we do not have a sufficient history to estimate the expected term, we use the simplified method as described in SAB Topic 14.D.2 for estimating the expected term. The simplified method is based on the average of the vesting tranches and the contractual life of each grant. Because there was no public market for our common stock prior to our initial public offering, we lacked company-specific historical and implied volatility information. Therefore, we used the historical volatility of a representative group of public biotechnology and life sciences companies with similar characteristics to us. In 2012, subsequent to our initial public offering, we began to consider including our own historical volatility, based on future expectations. We have not paid and do not anticipate paying cash dividends on our shares of common stock; therefore, the expected dividend yield is assumed to be zero. We also recognize compensation expense for only the portion of options that are expected to vest. Accordingly, we have estimated expected forfeitures of stock options based on our historical forfeiture rate, adjusted for known trends, and used these rates in developing a future forfeiture rate.

We have also granted performance-based restricted stock units (RSUs) with terms that allow the recipients to vest in a specific number of shares based upon the achievement of performance-based milestones as specified in the grants. Share-based compensation expense associated with these performance-based RSUs is recognized if the performance condition is considered probable of achievement using management's best estimates of the time to vesting for the achievement of the performance-based milestones. If the actual achievement of the performance-based milestones varies from our estimates, share-based compensation expense could be materially different than what is recorded in the period. The cumulative effect on current and prior periods of a change in the estimated time to vesting for performance-based RSUs will be recognized as compensation cost in the period of the revision, and recorded as a change in estimate.

While the assumptions used to calculate and account for share-based compensation awards represent management's best estimates, these estimates involve inherent uncertainties and the application of management's judgment. As a result, if revisions are made to our underlying assumptions and estimates, our share-based compensation expense could vary significantly from period to period.

As of December 31, 2014, there was approximately $13.9 million and $1.8 million of unrecognized stock-based compensation, net of estimated forfeitures, related to stock options and restricted stock units, respectively, which are expected to be recognized over weighted-average periods of 2.5 years and 1.1 years, respectively. There is no unrecognized stock-based compensation related to restricted stock

awards. The total unrecognized share-based compensation cost will be adjusted for future changes in estimated forfeitures. See Notes 2 and 7 to our consolidated financial statements located in this Annual Report on Form 10-K for further discussion of share-based compensation.

RESULTS OF OPERATIONS

In December 2012, Verastem Securities Company, our wholly owned subsidiary, was incorporated. All financial information presented has been consolidated and includes the accounts of our wholly owned subsidiary. All intercompany balances and transactions have been eliminated in consolidation.

Comparison of the Year Ended December 31, 2014 to the Year Ended December 31, 2013

Research and development expense. Research and development expense for the year ended December 31, 2014 (2014 Period) was $35.4 million compared to $25.9 million for the year ended December 31, 2013 (2013 Period). The $9.5 million increase from the 2013 Period to the 2014 Period was primarily related to an increase of $7.6 million in CRO expense for outsourced biology, chemistry, development and clinical services, which includes our clinical trial costs, a $1.4 million increase in personnel costs primarily due to increased headcount, an increase of approximately $528,000 in occupancy and maintenance expenses partially due to the relocation to our new facility, an approximate $412,000 net increase in license fees related to the Encarta asset purchase partially offset by the Poniard and S*Bio milestones incurred in the 2013 Period, an increase of approximately $265,000 in consulting expense and an approximate $152,000 increase in stock-based compensation expense due to increased headcount. These increases were partially offset by a decrease in lab supplies of approximately $774,000 partially due to less activity as the Company transitioned to the new facility and to the increase in outsourced services.

General and administrative expense. General and administrative expense for the 2014 Period was $18.2 million compared to $15.5 million for the 2013 Period. The $2.7 million increase from the 2013 Period to the 2014 Period primarily resulted from an increase of $1.6 million in stock-based compensation expense primarily due to an increase in stock option grants, an increase in consulting fees of $1.0 million primarily related to preparation for potential commercialization, an increase of approximately $912,000 in personnel costs primarily due to an increase in salaries and headcount, and an increase of approximately $192,000 in occupancy expense partially due to the relocation to our new facility. These increases were partially offset by a net decrease in professional fees and other costs of $1.0 million.

Interest income. Interest income increased to approximately $242,000 for the 2014 Period from approximately $200,000 for the 2013 Period. This increase was primarily due to a higher average investment balance for the 2014 Period compared to the 2013 Period.

Comparison of the Year Ended December 31, 2013 to the Year Ended December 31, 2012

Research and development expense. Research and development expense for the year ended December 31, 2013 (2013 Period) was $25.9 million compared to $21.7 million for the year ended December 31, 2012 (2012 Period). The $4.2 million increase from the 2012 Period to the 2013 Period was primarily related to an increase of $4.9 million in CRO expense for outsourced biology, chemistry and development services, which includes our clinical trial costs, a $1.8 million increase in personnel costs primarily due to increased average headcount, an approximate $619,000 increase in stock-based compensation expense and an increase of approximately $368,000 in travel fees primarily due to increased travel associated with our clinical trials. These increases were partially offset by a decrease of $3.5 million in license fee expense related to our agreement with Pfizer, Inc.

General and administrative expense. General and administrative expense for the 2013 Period was $15.5 million compared to $10.5 million for the 2012 Period. The $5.0 million increase from the 2012 Period to the 2013 Period primarily resulted from an increase of $2.4 million in stock-based compensation expense associated with restricted stock units, an increase in professional fees and other costs of $1.5 million, an increase in consulting fees of approximately $514,000, an approximate $274,000 increase in corporate franchise taxes and an approximate $339,000 increase in personnel costs primarily due to increase in salaries and headcount.

Interest income. Interest income decreased to approximately $200,000 for the 2013 Period from approximately $246,000 for the 2012 Period. This decrease was primarily due to lower coupon rates on investments for the 2013 Period compared to the 2012 Period.

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, our initial public offering in February 2012, our follow-on offerings in July 2013 and January 2015 and sales of common stock under our at-the market equity offering program. As of December 31, 2014, we had received $68.1 million in net proceeds from the issuance of preferred stock and $126.1 million in net proceeds from our public offerings. As of December 31, 2014, we had approximately $92.7 million in cash, cash equivalents and investments. We primarily invest our cash, cash equivalents and investments in a U.S. Treasury money market fund, government-sponsored enterprise securities and corporate bonds and commercial paper.

Cash flows

The following table sets forth the primary sources and uses of cash for each of the periods set forth below (in thousands):

	Years Ended December 31,		
	2012	2013	2014
Net cash provided by (used in)			
Operating activities	$ (22,601)	$ (26,305)	$ (36,902)
Investing activities	(45,859)	(23,393)	43,140
Financing activities	57,602	58,491	8,774
Net increase (decrease) in cash and cash equivalents	$ (10,858)	$ 8,793	$ 15,012

Operating activities. The use of cash in all periods resulted from our net losses adjusted for non-cash charges and changes in the components of working capital. The increase in cash used in the years ended December 31, 2014 and 2013 compared to the previous year was primarily due to an increase in research and development expenses related to our ongoing clinical trials and development of our lead product candidates. We expect cash used in operating activities to continue to increase for the foreseeable future as we fund our increased research, development and clinical activities.

Investing activities. The cash provided by investing activities for the year ended December 31, 2014 includes $45.7 million in net maturities of investments offset by approximately $2.4 million of property and equipment purchases primarily related to our relocation of our corporate headquarters and laboratory facility. The cash used in investing activities for the year ended December 31, 2013 includes $23.3 million in net purchases of investments and approximately $56,000 of property and equipment purchases. The cash used in investing activities for the year ended December 31, 2012

reflects the net purchases of investments of $45.5 million and the purchase of approximately $310,000 of property and equipment.

Financing activities. The cash provided by financing activities for the year ended December 31, 2014 is primarily related to $9.5 million of net proceeds from our at-the-market equity offering program offset by $780,000 of cash used to settle our restricted stock liability. The cash provided by financing activities for the year ended December 31, 2013 is primarily related to $59.8 million of net proceeds from our follow-on offering offset by $1.3 million cash used to settle our restricted stock liability. The cash provided by financing activities for the year ended December 31, 2012 reflects the $57.6 million of net proceeds from our initial public offering not including costs incurred in 2011 related to the transaction.

In December 2013, we established an at-the-market equity offering program pursuant to which we are able to offer and sell up to $35 million of our common stock at then current market prices from time to time through Cantor Fitzgerald & Co., as sales agent. In November 2014, we commenced sales under this program. Through December 31, 2014, we sold 1,346,676 shares of common stock under this program with net proceeds of approximately $11.6 million, after deducting commissions and other offering expenses. Approximately $2.1 million of these net proceeds were received in January 2015.

In January 2015, we sold an additional 276,663 shares of common stock under the at-the-market equity offering program with net proceeds of approximately $2.5 million, after deducting commissions and other offering expenses.

In January 2015, we completed a follow-on offering in which we sold 8,337,500 shares of our common stock to the public at a price of $6.50 per share, including 1,087,500 shares issued pursuant to the exercise of the underwriters' option to purchase additional shares. The net proceeds from this offering were approximately $50.9 million, after deducting underwriting discounts and commissions.

Funding requirements

We have three product candidates currently in clinical trials. 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 registration-directed trial of VS-6063 in mesothelioma, including the initiation of associated studies in preparation for a possible NDA filing to the FDA and similar filings to other regulatory authorities;
- continue our other ongoing clinical trials with VS-6063, VS-5584 and VS-4718;
- initiate additional clinical trials for our product candidates;
- 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;
- acquire or in-license other products and technologies;
- hire additional clinical, development 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 our existing cash, cash equivalents and investments, including approximately $55.5 million of net proceeds received during January 2015 from the sale of our common stock, will enable us to fund our current operating plan and capital expenditure requirements into the first half of 2017. 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 rate of enrollment, results and cost of completing of registration-directed trial of VS-6063 in mesothelioma;
- assuming favorable clinical results, the cost, timing and outcome of our efforts to seek approval in mesothelioma in the United States and elsewhere in the world, including to fund the preparation and filing of regulatory submissions with the FDA and other regulatory agencies worldwide;
- assuming regulatory approval, the costs of future commercialization activities, including product sales, marketing, manufacturing and distribution, of VS-6063 in mesothelioma in the United States and elsewhere in the world, whether alone or through a third party;
- the scope, progress and, results of our other ongoing and potential future clinical trials;
- 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 and the costs of future commercialization activities for such 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 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, 2014:

(in thousands)	Total	2015	2016 - 2017	2018 - 2019	Thereafter
Operating lease obligations	$ 2,493	$ 497	$ 1,039	$ 957	$ —
License agreements(1)	—	—	—	—	—

(1) As discussed in Note 10 to the consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K, we are party to 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 $135,000 per year beginning in 2015 up to a maximum amount of $140,000 per year beginning in 2016, as well as reimburse certain patent costs 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 prior written notice to the licensor.

In July 2012, we entered into a License Agreement with Pfizer, Inc., ("Pfizer"), under which Pfizer granted us worldwide, exclusive rights to research, develop, manufacture and commercialize products containing certain of Pfizer's inhibitors of focal adhesion kinase (the "Products"), including VS-6063, for all therapeutic, diagnostic and prophylactic uses in humans. We have the right to grant sublicenses under the foregoing licensed rights, subject to certain restrictions. We are solely responsible, at our own expense, for the clinical development of the Products, which is to be conducted in accordance with an agreed-upon development plan. We are also responsible for all manufacturing and commercialization activities at our own expense. Pfizer was required to provide us with an initial quantity of clinical supply of one of the Products for an agreed upon price. We made a one-time cash payment to Pfizer in the amount of $1.5 million and issued 192,012 shares of our common stock. Pfizer is also eligible to receive up to $2 million in developmental milestones and up to an additional $125 million based on the successful attainment of regulatory and commercial sales milestones. Pfizer is also eligible to receive high single to mid double digit royalties on future net sales of Products. Our royalty obligations with respect to each Product in each country begin on the date of first commercial sale of the Product in that country, and end on the later of 10 years after the date of first commercial sale of the Product in that country or the date of expiration or abandonment of the last claim contained in any issued patent or patent application licensed by Pfizer to us that covers the Product in that country.

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 that we entered into in November 2011 relating to VS-4718 and certain other compounds, we paid an upfront license fee and agreed to pay Poniard milestone payments upon the achievement of specified development and regulatory milestones. In February 2014, we purchased the assets which were the subject of our license agreement with Poniard from Encarta, Inc. (Encarta), who had previously purchased those assets in 2013. In consideration for the assets, we issued 97,500 shares of common stock, a warrant to purchase 142,857 shares of common stock with an exercise price equal to $17.16 per share and paid $25,000. All existing obligations under the license agreement, including an achieved development milestone and an obligation to issue a warrant, were settled as part of this transaction. In connection with the asset purchase agreement, we also assumed the rights and obligations under the Scripps License Agreement. Pursuant to the Scripps License Agreement, we are obligated to pay Scripps potential product development milestone payments of up to an aggregate of $3,000,000 upon the achievement of specified development and regulatory milestones. In addition, we are obligated to pay Scripps low single-digit royalties as a percentage of net sales of licensed products, subject to adjustments in certain circumstances. Our obligation to pay royalties on net sales is on a country by country basis.

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, 2014, we had federal and state net operating loss carryforwards of $108.0 million and $106.9 million, respectively, which are available to reduce future taxable income. We also had federal tax credits of $2.3 million and state tax credits of approximately $842,000, which may be used to offset future tax liabilities. The net operating loss and tax credit carryforwards will expire at various dates through 2034. 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, 2014, we recorded a 100% valuation allowance against our net operating loss and tax credit carryforwards of approximately $48.3 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

Development Stage Entity

On June 10, 2014, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") 2014-10, which simplifies financial reporting for development stage entities by eliminating requirements specific to development stage entities. As a result, entities in a development stage no longer need to present inception-to-date information about statement of operations line items, cash flows, and equity transactions. Instead, the new guidance clarifies how these entities should tailor existing disclosures to explain the risks and uncertainties related to their activities. This update is effective for annual periods beginning after December 15, 2014, and early application is permitted for any annual or interim period for which the entity's financial statements have not yet been issued. We adopted this guidance prior to issuing the interim financial statements in the Q2 2014 Form 10-Q. The adoption of ASU 2014-10 impacted disclosure only and did not have any impact on financial position or results of operations.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk

We are exposed to market risk related to changes in interest rates. We had cash, cash equivalents and investments of $92.7 million and $123.7 million as of December 31, 2014 and 2013, respectively, consisting of cash, money market funds, United States Treasuries, government-sponsored enterprise securities and corporate bonds and commercial paper. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because most of our investments are interest bearing. 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 which may be denominated in foreign currencies. 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, 2014 and 2013, $1.6 million and approximately $790,000 of our total liabilities was denominated in currencies other than the functional currency, respectively.

Item 8. Consolidated Financial Statements and Supplementary Data

Our consolidated 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 Chief Executive Officer and our Chief Financial Officer evaluated the effectiveness of our disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, as of the end of the period covered by this report. Based on that evaluation, our Chief Executive Officer and our Chief Financial Officer concluded that our disclosure controls and procedures as of the end of the period covered by this report were effective.

Management's Report on Internal Control Over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over our financial reporting. Internal control over financial reporting is defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act as the process designed by, or under the supervision of, our Chief Executive Officer and our Chief Financial Officer, and effected by our board of directors, management and other personnel, to provide reasonable assurance regarding the reliability of our financial reporting and the preparation of our financial statements for external purposes in accordance with generally accepted accounting principles, and includes those policies and procedures that:

(1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of assets;

(2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures are being made only in accordance with the authorizations of management and directors; and

(3) provide reasonable assurance regarding the prevention or timely detection of unauthorized acquisition, use or disposition of assets that could have a material effect on our financial statements.

Under the supervision and with the participation of our management, including our Chief Executive Officer and our Chief Financial Officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting based on the framework provided in *Internal Control—Integrated Framework* issued by the Committee of Sponsoring Organizations of the Treadway Commission (1992 Framework). Based on this evaluation, our management concluded that our internal control over financial reporting was effective as of December 31, 2014.

Changes in Internal Control Over Financial Reporting

There has been no change in our internal control over financial reporting occurred during the fiscal quarter ended December 31, 2014 that has materially affected, or is reasonably likely to materially affect, the Company's internal control over financial reporting.

Item 9B. Other Information

None

PART III

Item 10. Directors, Executive Officers, and Corporate Governance

Other than the information regarding our executive officers provided in Part I of this report under the heading "Business—Executive Officers of the Registrant," the information required to be furnished pursuant to this item is incorporated herein by reference to our definitive proxy statement for the 2015 Annual Meeting of the Stockholders.

Item 11. Executive Compensation

The information required by this Item 11 is incorporated herein by reference from our definitive proxy statement for the 2015 Annual Meeting of Stockholders.

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

The information required by this Item 12 is incorporated herein by reference from our definitive proxy statement for the 2015 Annual Meeting of Stockholders.

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

The information required by this Item 13 is incorporated herein by reference from our definitive proxy statement for the 2015 Annual Meeting of Stockholders.

Item 14. Principal Accountant Fees and Services

The information required by this Item 14 is incorporated herein by reference from our definitive proxy statement for the 2015 Annual Meeting of Stockholders.

PART IV

Item 15. Exhibits and Financial Statement Schedules

Consolidated Financial Statements

See Part II, Item 8 for the Financial Statements required to be included in this Annual Report on Form 10-K.

Consolidated Financial Statement Schedules

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

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 on this 10th day of March 2015.

VERASTEM, INC.

By: /s/ ROBERT FORRESTER

Robert Forrester
Chief Executive Officer

Pursuant to the requirements of the Securities Exchange 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/ ROBERT FORRESTER Robert Forrester	Chief Executive Officer and Director (Principal executive officer)	March 10, 2015
/s/ JOHN B. GREEN, CPA John B. Green, CPA	Chief Financial Officer (Principal financial officer and Principal accounting officer)	March 10, 2015
/s/ TIMOTHY BARBERICH Timothy Barberich	Director	March 10, 2015
/s/ PAUL A. FRIEDMAN Paul A. Friedman	Director	March 10, 2015
/s/ MICHAEL KAUFFMAN, M.D., PH.D. Michael Kauffman, M.D., Ph.D.	Director	March 10, 2015
/s/ ALISON LAWTON Alison Lawton	Director	March 10, 2015
/s/ S. LOUISE PHANSTIEL S. Louise Phanstiel	Director	March 10, 2015
/s/ STEPHEN SHERWIN, M.D. Stephen Sherwin, M.D.	Director	March 10, 2015

Signature	Title	Date
/s/ HENRI TERMEER Henri Termeer	Director	March 10, 2015
/s/ CHRISTOPH WESTPHAL, M.D., PH.D. Christoph Westphal, M.D., Ph.D.	Director	March 10, 2015

Verastem, Inc.

CONSOLIDATED FINANCIAL STATEMENTS

CONTENTS

Verastem, Inc.

Report of Independent Registered Public Accounting Firm

The Board of Directors and Stockholders of
Verastem, Inc.

We have audited the accompanying consolidated balance sheets of Verastem, Inc. as of December 31, 2013 and 2014, and the related consolidated statements of operations and comprehensive loss, stockholders' (deficit) equity and cash flows for each of the three years in the period ended December 31, 2014. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements and schedule 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 audit 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 consolidated financial position of Verastem, Inc. as of December 31, 2013 and 2014, and the consolidated results of its operations and its cash flows for each of the three years in the period ended December 31, 2014 in conformity with U.S. generally accepted accounting principles.

/s/ Ernst & Young

Boston, Massachusetts
March 10, 2015

Verastem, Inc.

CONSOLIDATED BALANCE SHEETS

(in thousands, except per share amounts)

	December 31,	
	2013	2014
Assets		
Current assets:		
Cash and cash equivalents	$ 18,889	$ 33,901
Short-term investments	82,423	58,774
Restricted cash	86	—
Prepaid expenses and other current assets	557	2,641
Total current assets	101,955	95,316
Property and equipment, net	631	2,825
Long-term investments	22,344	—
Restricted cash	—	203
Other assets	331	305
Total assets	$ 125,261	$ 98,649
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 2,760	$ 3,216
Accrued expenses	4,327	5,519
Liability classified stock-based compensation awards	717	469
Total current liabilities	7,804	9,204
Other liabilities	—	677
Liability for shares subject to repurchase	11	2
Stockholders' equity:		
Convertible preferred stock, $0.0001 par value; 5,000 shares authorized, no shares issued and outstanding	—	—
Common stock, $0.0001 par value; 100,000 shares authorized, 25,328 and 27,259 shares issued and outstanding at December 31, 2013 and 2014, respectively	3	3
Additional paid-in capital	205,068	229,770
Accumulated other comprehensive income	28	11
Accumulated deficit	(87,653)	(141,018)
Total stockholders' equity	117,446	88,766
Total liabilities and stockholders' equity	$ 125,261	$ 98,649

See accompanying notes.

Verastem, Inc.

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(in thousands, except per share amounts)

	Years Ended December 31,		
	2012	2013	2014
Operating expenses:			
Research and development	$ 21,712	$ 25,930	$ 35,448
General and administrative	10,518	15,472	18,159
Total operating expenses	32,230	41,402	53,607
Loss from operations	(32,230)	(41,402)	(53,607)
Interest income	246	200	242
Net loss	(31,984)	(41,202)	(53,365)
Accretion of preferred stock	(6)	—	—
Net loss applicable to common stockholders	$ (31,990)	$ (41,202)	$ (53,365)
Net loss per share applicable to common stockholders	$ (1.70)	$ (1.82)	$ (2.07)
Weighted-average number of common shares used in net loss per share applicable to common stockholders—basic and diluted	18,765	22,680	25,804
Net loss	$ (31,984)	$ (41,202)	$ (53,365)
Unrealized gains (losses) on available-for-sale securities	24	6	(17)
Comprehensive loss	$ (31,960)	$ (41,196)	$ (53,382)

See accompanying notes.

Verastem, Inc.

CONSOLIDATED STATEMENTS OF REDEEMABLE CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' (DEFICIT) EQUITY

(in thousands, except share data)

	Redeemable convertible preferred stock		Common stock		Additional paid-in capital	Accumulated other comprehensive (loss) income	Accumulated Deficit	Totals stockholders' (deficit) equity
	Shares	Amount	Shares	Amount				
Balance at December 31, 2011	**41,092,825**	**$ 68,141**	**1,558,588**	**$ 1**	**$ 1,702**	**$ (2)**	**$ (14,467)**	**$ (12,766)**
Net loss	—	—	—	—	—	—	(31,984)	(31,984)
Unrealized gain on available-for-sale marketable securities	—	—	—	—	—	24	—	24
Accretion of redeemable convertible preferred stock to redemption value	—	6	—	—	(6)	—	—	(6)
Conversion of redeemable convertible preferred stock into common stock	(41,092,825)	(68,147)	11,740,794	1	68,146	—	—	68,147
Issuance of common stock, net of issuance costs of $1,984	—	—	6,325,000	—	56,838	—	—	56,838
Issuance of common stock in exchange for license	—	—	192,012	—	1,957	—	—	1,957
Vesting of restricted stock	—	—	542,421	—	16	—	—	16
Issuance of common stock resulting from exercise of stock options	—	—	5,395	—	3	—	—	3
Obligation to issue a warrant	—	—	—	—	837	—	—	837
Stock-based compensation expense	—	—	—	—	7,400	—	—	7,400
Balance at December 31, 2012	**—**	**$ —**	**20,364,210**	**$ 2**	**$ 136,893**	**$ 22**	**$ (46,451)**	**$ 90,466**
Net loss	—	—	—	—	—	—	(41,202)	(41,202)
Unrealized gain on available-for-sale marketable securities	—	—	—	—	—	6	—	6
Issuance of common stock, net of issuance costs of $235	—	—	4,255,000	1	59,759	—	—	59,760
Vesting of restricted stock	—	—	417,718	—	9	—	—	9
Issuance of common stock resulting from exercise of stock options	—	—	81,795	—	33	—	—	33
Issuance of common stock resulting from vesting of restricted stock units and payment of tax withholdings	—	—	209,727	—	(1,302)	—	—	(1,302)
Stock-based compensation expense	—	—	—	—	9,676	—	—	9,676
Balance at December 31, 2013	**—**	**$ —**	**25,328,450**	**$ 3**	**$ 205,068**	**$ 28**	**$ (87,653)**	**$ 117,446**
Net loss	—	—	—	—	—	—	(53,365)	(53,365)
Unrealized loss on available-for-sale marketable securities	—	—	—	—	—	(17)	—	(17)
Issuance of common stock, net of issuance costs of $43	—	—	1,346,676	—	11,646	—	—	11,646
Vesting of restricted stock	—	—	321,287	—	9	—	—	9

Issuance of common stock resulting from exercise of stock options	—	—	30,451	—	20	—	—	20
Issuance of common stock resulting from vesting of restricted stock units and payment of tax withholdings	—	—	135,008	—	(780)	—	—	(780)
Shares issued for technology rights	—	—	97,500	—	1,447	—	—	1,447
Stock-based compensation expense	—	—	—	—	12,360	—	—	12,360
Balance at December 31, 2014	**— $**	**—**	**27,259,372 $**	**3 $**	**229,770 $**	**11 $**	**(141,018)$**	**88,766**

See accompanying notes.

Verastem, Inc.

CONSOLIDATED STATEMENTS OF CASH FLOWS

(in thousands)

	Years Ended December 31,		
	2012	2013	2014
Operating activities			
Net loss	$ (31,984)	$ (41,202)	$ (53,365)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	207	236	427
Stock-based compensation expense	7,400	9,676	12,360
Amortization of premiums and discounts on available-for-sale marketable securities	—	—	290
Non-cash expense related to purchase of technology rights	—	—	1,197
Non-cash expense related to acquisition of license	1,957	—	—
Change in fair value of obligation to issue a warrant	431	—	—
Loss on disposal of fixed assets	—	—	4
Changing in operating assets and liabilities:			
Prepaid expenses and other current assets	(376)	(382)	27
Accounts payable	(425)	912	287
Accrued expenses and other liabilities	189	3,738	2,119
Liability classified stock-based compensation awards	—	717	(248)
Net cash used in operating activities	(22,601)	(26,305)	(36,902)
Investing activities			
Purchases of property and equipment	(310)	(56)	(2,429)
Purchases of investments	(154,028)	(118,190)	(39,361)
Maturities of investments	108,479	94,853	85,047
Increase in restricted cash	—	—	(117)
Net cash (used in) provided by investing activities	(45,859)	(23,393)	43,140
Financing activities			
Proceeds from the exercise of stock options	3	33	20
Net proceeds from the issuance of common stock and restricted common stock	57,599	59,760	9,534
Cash used to settle restricted stock liability	—	(1,302)	(780)
Net cash provided by financing activities	57,602	58,491	8,774
(Decrease) increase in cash and cash equivalents	(10,858)	8,793	15,012
Cash and cash equivalents at beginning of period	20,954	10,096	18,889
Cash and cash equivalents at end of period	$ 10,096	$ 18,889	$ 33,901
Supplemental disclosure of non-cash investing and financing activities			
Accretion of redeemable convertible preferred stock to redemption value	$ 6	$ —	$ —
Conversion of redeemable convertible preferred stock upon initial public offering	$ 68,147	$ —	$ —
Reclassification of obligation to issue warrant from liabilities to equity	$ 837	$ —	$ —
Proceeds from the issuance of common stock included in prepaid expenses and other current assets	$ —	$ —	$ 2,085
Purchases of property and equipment in accounts payable	$ —	$ —	$ 196

See accompanying notes.

Verastem, Inc.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

1. Nature of business

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. 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 and clinical studies of its most advanced product candidates.

The Company is subject to a number of risks similar to other life science companies, 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, 2014, the Company had an accumulated deficit of $141.0 million. The Company expects to continue to incur operating losses in future periods. The Company had cash, cash equivalents and investments of $92.7 million as of December 31, 2014. The Company believes that its existing cash, cash equivalents and investments, including $55.5 million of net proceeds received in January 2015 from the sale of common stock, will be sufficient to fund its current operating plan and capital expenditure requirements for at least the next twelve months.

2. Significant accounting policies

Basis of consolidation

Effective December 2012, Verastem Securities Company was incorporated as a wholly owned subsidiary of the Company and all financial information presented has been consolidated and includes the accounts of the Company and its wholly owned subsidiary. All intercompany balances and transactions have been eliminated in consolidation.

Use of estimates

The preparation of the Company's financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. On an ongoing basis, management evaluates its estimates, including estimates related to accruals and stock-based compensation expense. The Company bases its estimates on historical experience and other market-specific or other relevant assumptions that it believes to be reasonable. 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 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.

Verastem, Inc.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

2. Significant accounting policies (Continued)

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* (Practice Aid), to estimate the fair value of its common stock. Each of the Company's common stock valuations methodology included estimates and assumptions that required its judgment. These estimates included 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. The Company operates in only one geographic area.

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, government-sponsored enterprise securities and corporate bonds and commercial paper. Cash equivalents are reported at fair value.

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 prioritizes valuation inputs based on the observable nature of those inputs. The fair value hierarchy applies only to the valuation inputs used in determining the reported fair value of the investments and is not a measure of the investment credit quality. The hierarchy defines three levels of valuation inputs:

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

Verastem, Inc.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

2. Significant accounting policies (Continued)

The following table presents information about the Company's financial assets and liabilities that have been measured at fair value at December 31, 2014 and indicates the fair value hierarchy of the valuation inputs utilized to determine 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	$ 32,140	$ 32,140	$ —	$ —
Short-term investments	58,774	—	58,774	—
Total financial assets	**$ 90,914**	**$ 32,140**	**$ 58,774**	**$ —**
Financial liabilities				
Liability classified stock-based compensation awards	$ 469	$ 469	$ —	$ —
Total financial liabilities	**$ 469**	**$ 469**	**$ —**	**$ —**

The following table presents information about the Company's financial assets and liabilities that have been measured at fair value at December 31, 2013 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	$ 17,000	$ 17,000	$ —	$ —
Short-term investments	82,423	—	82,423	—
Long-term investments	22,344	—	22,344	—
Total financial assets	**$ 121,767**	**$ 17,000**	**$ 104,767**	**$ —**
Financial liabilities				
Liability classified stock-based compensation awards	$ 717	$ 717	$ —	$ —
Total financial liabilities	**$ 717**	**$ 717**	**$ —**	**$ —**

In connection with the license agreement with Poniard Pharmaceuticals Inc. (Poniard), as discussed in Note 10, the Company was obligated to issue a warrant to Poniard for the purchase of the Company's common stock upon the first patient dosing using a product licensed under the agreement with Poniard; such warrant would have a three year term from the date of issuance. Prior to the Company's initial public offering, the exercise price of the warrant would be equal to the fair value of the common stock on the date of the most recent preferred stock financing prior to the issuance of the warrant. Upon the completion of the Company's initial public offering in January 2012, the exercise price of the warrant was 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 was settled and a warrant was issued in February 2014.

Verastem, Inc.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

2. Significant accounting policies (Continued)

Prior to January 2012, the obligation to issue the warrant was a level 3 liability because its value measurement was based, in part, on significant inputs not observed in the market and reflects the Company's assumptions as to the expected warrant exercise price and the expected volatility of the Company's common stock. The obligation to issue the warrant was initially recorded at fair value and, prior to the Company's initial public offering, was revalued at the end of each reporting period, with the change in the fair value reported in research and development expense within the statement of operations. Upon the completion of the Company's initial public offering, the obligation to issue the warrant met the definition of an equity-classified derivative instrument since the remaining variable inputs were consistent with those in a fixed for fixed forward option agreement, and was therefore revalued as of January 26, 2012 with the change in fair value reported in research and development expense within the statement of operations. The fair value of the obligation to issue the warrant was then reclassified from liabilities to additional paid- in-capital on the Company's consolidated balance sheet.

Investments

Investments and cash equivalents consist of investments in money market accounts, government-sponsored enterprise securities and corporate bonds and commercial paper of publicly traded companies 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 consolidated balance sheets. Investments are classified as long-term assets on the consolidated 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 income (loss), which is a separate component of stockholders' equity (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 available evidence, the unrealized loss is transferred from other comprehensive loss to the statement of operations. There were no other-than-temporary declines in fair value of short-term or long-term investments for the years ended December 31, 2012, 2013 and 2014. 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 for the years ended December 31, 2012, 2013 and 2014.

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, 2013 and 2014, 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.

Verastem, Inc.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

2. Significant accounting policies (Continued)

As of December 31, 2014, cash, cash equivalents and investments included (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Cash and cash equivalents:				
Cash and cash equivalents	$ 33,901	$ —	$ —	$ 33,901
Total cash and cash equivalents	$ 33,901	$ —	$ —	$ 33,901
Investments:				
Government-sponsored enterprise securities (due within 1 year)	$ 3,700	$ —	$ —	$ 3,700
Corporate bonds and commercial paper (due within 1 year)	55,063	18	(7)	55,074
Total investments	$ 58,763	$ 18	$ (7)	$ 58,774
Total cash, cash equivalents, and investments	**$ 92,664**	**$ 18**	**$ (7)**	**$ 92,675**

As of December 31, 2013, cash, cash equivalents and investments included (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Cash and cash equivalents:				
Cash and cash equivalents	$ 18,889	$ —	$ —	$ 18,889
Total cash and cash equivalents	$ 18,889	$ —	$ —	$ 18,889
Investments:				
Government-sponsored enterprise securities (due within 1 year)	$ 30,652	$ 12	$ —	$ 30,664
Government-sponsored enterprise securities (due within 1 - 2 years)	4,001	2	—	4,003
Corporate bonds and commercial paper (due within 1 year)	51,735	30	(6)	51,759
Corporate bonds (due within 1 - 2 years)	18,351	2	(12)	18,341
Total investments	$ 104,739	$ 46	$ (18)	$ 104,767
Total cash, cash equivalents, and investments	**$ 123,628**	**$ 46**	**$ (18)**	**$ 123,656**

Concentrations of credit risk and off-balance sheet risk

Cash and cash equivalents and investments are financial instruments that potentially subject the Company to concentrations of credit risk. As of December 31, 2014, the Company's cash, cash equivalents and investments were deposited at two financial institutions. 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.

Verastem, Inc.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

2. Significant accounting policies (Continued)

Property and equipment

Property and equipment consists of laboratory equipment, office furniture, computer equipment and leasehold improvements. 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
Leasehold Improvements	Lesser of useful life or life of lease

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 recoverable. Recoverability is measured by comparison of the asset's book value to future net undiscounted cash flows that the assets are expected to generate. If such assets are considered to be impaired, the impairment to be recognized is measured by the amount by which the book value of the assets exceed their fair value, which is measured based on the projected discounted future net cash flows arising from the assets. No such impairment losses have been recorded through December 31, 2014.

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 product candidates. 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, clinical trial sites, 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 equipment, and laboratory 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

Verastem, Inc.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

2. Significant accounting policies (Continued)

date fair value of each stock-based award is estimated using the Black-Scholes option pricing model and is expensed on a straight-line basis over the vesting period. Awards subject to performance based vesting requirements are expensed utilizing an accelerated attribution model if achievement of the performance criteria is determined to be probable.

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. Stock option awards are revalued at each reporting date and upon vesting using the Black-Scholes option pricing model and are expensed on a straight-line basis over the vesting period.

Stock-based compensation awards which allow for greater than the minimum statutory tax withholdings are classified as liabilities. These awards are revalued at each reporting date and are expensed on a straight-line basis over the vesting period. Upon settlement, the awards are revalued and the amounts are reclassified to additional paid-in capital. Shares are withheld to cover the tax withholding and amounts paid to settle the tax liability are recorded as a reduction of additional paid-in capital.

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 outstanding stock options, restricted stock units, unvested restricted stock and the warrant issued in 2014 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. All potentially dilutive securities were excluded from the calculation of diluted net loss per share as the securities were anti-dilutive for all periods presented.

Verastem, Inc.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

2. Significant accounting policies (Continued)

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

	Years Ended December 31,		
	2012	2013	2014
Outstanding stock options	1,424,241	2,388,062	4,206,440
Restricted stock units	899,204	529,850	293,747
Outstanding warrants	—	—	142,857
Unvested restricted stock	747,000	329,282	7,995
	3,070,445	3,247,194	4,651,039

Recent Accounting Pronouncements

Development Stage Entity

On June 10, 2014, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") 2014-10, which simplifies financial reporting for development stage entities by eliminating requirements specific to development stage entities. As a result, entities in a development stage will no longer need to present inception-to-date information about statement of operations line items, cash flows, and equity transactions. Instead, the new guidance clarifies how these entities should tailor existing disclosures to explain the risks and uncertainties related to their activities. This update is effective for annual periods beginning after December 15, 2014, and early application is permitted for any annual or interim period for which the entity's financial statements have not yet been issued. The Company adopted this guidance prior to issuing the interim financial statements in the Q2 2014 Form 10-Q. The adoption of ASU 2014-10 impacted disclosure only and did not have any impact on financial position or results of operations.

Going Concern

In August 2014, the FASB issued ASU 2014-15, *Presentation of Financial Statements—Going Concern: Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern* (Subtopic 205-40). ASU 2014-15 requires management to assess an entity's ability to continue as a going concern every reporting period, and provide certain disclosures if management has substantial doubt about the entities ability to operate as a going concern, or an express statement if not, by incorporating and expanding upon certain principles that are currently in U.S. auditing standards. ASU 2014-15 is effective for the annual period ending after December 15, 2016, and for annual periods and interim periods thereafter. Early application is permitted. The adoption of ASU 2014-15 is not expected to have an impact on our financial position or results of operations.

Verastem, Inc.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

3. Property and equipment

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

	December 31, 2013	December 31, 2014
Laboratory equipment	$ 1,035	$ 1,040
Computer equipment	27	225
Furniture	44	325
Leasehold improvements	38	2,096
	1,144	3,686
Less: accumulated depreciation	(513)	(861)
	$ 631	$ 2,825

Total depreciation expenses amounted to $207,000, $236,000 and $427,000 for the years ended December 31, 2012, 2013 and 2014, respectively.

4. Prepaid expenses and other current assets

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

	December 31, 2013	December 31, 2014
Receivable from unsettled at-the-market equity transactions	$ —	$ 2,085
Prepaid contract research organization costs	411	238
Receivable related to tenant improvement allowance	—	150
Interest receivable	123	113
Prepaid other	23	55
	$ 557	$ 2,641

5. Accrued expenses

Accrued expenses are as follows (in thousands):

	December 31, 2013	December 31, 2014
Contract research organization costs	$ 1,918	$ 3,049
Compensation and related benefits	1,687	1,990
License milestones	360	45
Professional fees	237	233
Deferred rent	38	144
Other	87	58
	$ 4,327	$ 5,519

Verastem, Inc.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

6. Common stock

As of December 31, 2013 and 2014, the Company had reserved the following shares of common stock for the issuance of common stock for vested restricted stock units, the exercise of stock options, and an outstanding warrant (in thousands):

	December 31, 2013	December 31, 2014
Shares reserved under equity compensation plans	4,330	4,508
Shares reserved for inducement grants	—	750
Shares reserved for outstanding warrants	—	143
	4,330	5,401

Each share of common stock is entitled to one vote. 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.

Initial public offering

In February 2012, the Company closed the initial public offering (IPO) of its common stock pursuant to a registration statement on Form S-1, as amended. An aggregate of 6,325,000 shares of common stock registered under the registration statement were sold at a price of $10.00 per share, including the over-allotment option. Net proceeds of the IPO were $56.8 million. All shares of the Company's Redeemable Convertible Preferred Stock were converted into 11,740,794 shares of common stock as part of the IPO.

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.

At-the-market equity offering program

In December 2013, the Company established an at-the-market equity offering program pursuant to which it was able to offer and sell up to $35 million of its common stock at then current market prices from time to time. In November 2014, the Company commenced sales under this program. Through December 31, 2014, the Company sold 1,346,676 shares of common stock under this program with net proceeds of approximately $11.6 million, after deducting commissions and other offering expenses. Approximately $2.1 million of these net proceeds were received in January 2015.

Warrant

In February 2014, in connection with the acquisition of intellectual property rights from Encarta, Inc., the Company issued a warrant to purchase 142,857 shares of common stock exercisable at a price of $17.16 per share that expires 3 years from the issuance date.

Verastem, Inc.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

7. Stock-based compensation

Stock-based compensation expense as reflected in the Company's consolidated statements of operations and comprehensive loss was as follows (in thousands):

	Year Ended December 31,		
	2012	2013	2014
Research and development	$ 2,956	$ 3,575	$ 3,727
General and administrative	4,444	6,818	8,385
Total stock-based compensation expense	$ 7,400	$ 10,393	$ 12,112

Of the $10.4 million of stock-based compensation expense recorded during the year ended December 31, 2013, approximately $9.7 million was recorded to additional paid-in capital and approximately $717,000 was recorded to a change in liability classified awards. Of the $12.1 million of stock-based compensation expense recorded during the year ended December 31, 2014, approximately $12.3 million was recorded to additional paid-in capital and approximately $(248,000) was recorded to a change in liability classified awards.

The Company has awards outstanding under two equity compensation plans, the 2012 Incentive Plan (the "2012 Plan") and the 2010 Equity Incentive Plan (the "2010 Plan"). Terms of stock award agreements, including vesting requirements, are determined by the board of directors, subject to the provisions of the individual plans. To date, most options granted by the Company vest twenty five percent (25%) one year from vesting start date and six and a quarter percent (6.25%) for each successive three-month period, thereafter (subject to acceleration of vesting in the event of certain change of control transactions) and are exercisable from the date of grant for a period of ten years.

2012 Incentive Plan

The 2012 Plan became effective immediately upon the closing of the Company's IPO in February 2012. Upon effectiveness of the 2012 Plan, the Company ceased making awards under the 2010 Plan. The 2012 Plan allows the Company to grant awards for up to 3,428,571 shares of common stock plus the number of shares of common stock available for grant under the 2010 Plan as of the effectiveness of the 2012 Plan (which was an additional 30,101 shares) plus that number of shares of common stock related to awards outstanding under the 2010 Plan which terminate by expiration, forfeiture, cancellation or otherwise. 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. On January 1, 2013 and January 1, 2014, the number of shares available for issuance under the 2012 Plan increased by 844,448 and 1,026,309 respectively for shares under this provision. Awards under the 2012 Plan may include the following award types: incentive stock options, nonqualified stock options, stock appreciation rights, restricted stock awards, restricted stock units (RSUs), other stock-based or cash-based awards and any combination of the foregoing. As of December 31, 2014, under the 2012 Plan, the Company has granted stock options for 4,116,629 shares of common stock, of which 151,789 have been forfeited and restricted stock units for 909,918 shares of common stock, of which 74,515 have been forfeited. The exercise price of each option has been equal

Verastem, Inc.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

7. Stock-based compensation (Continued)

to the closing price of a share of our common stock on the grant date. On January 1, 2015, the number of shares available for issuance under the 2012 Plan increased by 1,081,045.

Inducement Plan

In December 2014, the Company established an inducement award program (in accordance with NASDAQ Listing Rule 5635(c)(4)) under which it may grant non-statutory stock options to purchase up to an aggregate of 750,000 shares of common stock to new or prospective employees as inducement to enter into employment with the Company. The program is governed by the terms of the 2012 Plan but does not fall under the 2012 Plan. No awards have been granted under this program as of December 31, 2014.

Restricted Common Stock

In connection with the Company's formation, the founders purchased an aggregate of 2,857,138 shares of Company's common stock at fair value on the date of issuance. The shares were issued subject to restricted stock agreements between the Company and each founder, which allow the Company, at its discretion, to repurchase unvested shares if the founder's relationship with the Company is terminated. Under these agreements, twenty five percent (25%) of the shares vested immediately and the remaining seventy-five percent (75%) of shares vest ratably in quarterly installments over the subsequent four years.

The Company records stock-based compensation expense for the common stock subject to repurchase, or restricted common stock grants, based on the grant date fair value for employees and the reporting date and upon vesting fair value for non-employees. The fair value of the award is considered the intrinsic value as of each measurement date. All of the restricted shares were issued at a purchase price equal to the fair value of the common stock on the date of issuance. The Company recorded stock-based compensation expense associated with restricted common stock grants of approximately $3.3 million, $3.4 million and $1.7 million for the years ended December 31, 2012, 2013 and 2014, respectively.

A summary of the Company's restricted stock activity and related information is as follows:

	Shares	Weighted-average grant date fair value per share
Unvested at December 31, 2013	329,282	$ 0.034
Vested	(321,287)	$ 0.028
Unvested at December 31, 2014	7,995	$ 0.28

No restricted stock was granted during the years ended December 31, 2012, 2013 and 2014. The total fair value of shares vested during the years ended December 31, 2012, 2013 and 2014 was approximately, $3.4 million, $3.3 million and $2.2 million, respectively. As of December 31, 2014, there was no unrecognized stock-based compensation expense related to unvested restricted stock.

Verastem, Inc.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

7. Stock-based compensation (Continued)

Restricted Stock Units

A summary of the Company's restricted stock units ("RSU") and related information is as follows:

	Shares	Weighted-average grant date fair value per share
Unvested at December 31, 2013	529,850	$ 10.78
Vested	(210,873)	$ 10.52
Forfeited	(25,230)	$ 10.32
Unvested at December 31, 2014	293,747	$ 10.54

The weighted-average grant date fair value of restricted stock units granted during the year ended December 31, 2012 was $10.62. No restricted stock units were granted during the years ended December 31, 2013 and 2014. The total fair value of restricted stock units vested during the years ended December 31, 2013 and 2014 was $3.4 million and $2.2 million, respectively. No restricted stock units vested during the year ended December 31, 2012. As of December 31, 2014, there was $1.8 million of total unrecognized stock-based compensation expense related to unvested RSUs granted under the 2012 Plan. The expense is expected to be recognized over a weighted-average period of 1.1 years.

During 2012, the Company issued a restricted stock unit for 103,306 shares to an employee. The award vests up to 25% per year based on the achievement of stated objectives. The objectives related to 2013 were established on January 15, 2013 and the objectives were determined to be met on December 17, 2013. The Company recorded $294,000 of stock-based compensation expense during the year ended December 31, 2013 based on the achievement of the stated objectives. The objectives related to 2014 were established on January 7, 2014 and the objectives were determined to be met on January 8, 2015. The Company recorded $158,000 of stock-based compensation expense during the year ended December 31, 2014 based on the achievement of the stated objectives.

The objectives related to 2015 were not determined as of December 31, 2014 and therefore the Company did not have sufficient information to determine if the achievement of the objectives was probable, and therefore no expense related to these objectives has been recorded as of December 31, 2014.

During the first quarter of 2013, the Company amended the terms of certain RSUs related to a total of 697,060 shares of common stock to allow for tax withholdings greater than the minimum required statutory withholding amount. As a result of this change in the terms of the awards, the outstanding RSUs are considered to be liability instruments. As a result of this modification, the Company records a liability for the fair value of the awards as of each reporting date with the change in fair value recorded through the statement of operations. The Company will record stock-based compensation expense equal to the greater of the original grant date fair value of the awards or the settlement date fair value. During the year ended December 31, 2013 and 2014, the Company deposited with tax authorities $1.3 million and approximately $780,000, respectively, to settle the tax liability for awards that settled during the respective periods. The liability related to these awards of approximately $717,000 and $469,000 is recorded as liability classified stock-based compensation awards on the consolidated balance sheet as of December 31, 2013 and 2014, respectively.

Verastem, Inc.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

7. Stock-based compensation (Continued)

Stock Options

A summary of the Company's stock option activity and related information follows:

	Shares	Weighted-average exercise price per share	Weighted-average remaining contractual term (years)	Aggregate intrinsic value (in thousands)
Outstanding at December 31, 2013	2,388,062	$ 8.62	8.8	$ 6,768
Granted	1,956,415	$ 12.39		
Exercised	(30,451)	$ 0.68		
Forfeited	(107,586)	$ 10.47		
Outstanding at December 31, 2014	4,206,440	$ 10.38	8.4	$ 2,593
Vested at December 31, 2014	1,728,322	$ 8.88	7.8	$ 2,067
Vested and expected to vest at December 31, 2014(1)	3,955,095	$ 10.35	8.4	$ 2,567

(1) This represents the number of vested options as of December 31, 2014, plus the number of unvested options expected to vest as of December 31, 2014, based on the unvested options at December 31, 2014, adjusted for the estimated forfeiture rate.

The fair value of each employee stock option was estimated at the date of grant using a Black-Scholes option-pricing model with the following assumptions:

	Year Ended December 31,		
	2012	2013	2014
Risk-free interest rate	1.14%	1.14%	1.99%
Volatility	76%	75%	81%
Dividend yield	—	—	—
Expected term (years)	6.0	6.0	6.0

The Company uses the simplified method as prescribed by the Securities and Exchange Commission Staff Accounting Bulletin Topic 14.D.2 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. 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 public biotechnology and life sciences companies with similar characteristics to the Company, including early stage of product development and therapeutic focus. The Company also considers its own historical volatility. The risk-free interest rate is based on a treasury instrument whose term is consistent with the expected term of the stock options. Management assesses expected forfeitures based on the experience of the Company coupled with comparison to data from the representative group of companies and recognizes compensation costs only for those equity awards expected to vest.

Verastem, Inc.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

7. Stock-based compensation (Continued)

The Company recorded stock-based compensation expense associated with employee stock options of $1.6 million, $6.8 million and $9.9 million for the years ended December 31, 2012, 2013 and 2014, respectively. The weighted-average grant date fair value of options granted in the years ended December 31, 2012, 2013 and 2014 was $6.03, $6.55 and $8.61 per share, respectively.

At December 31, 2014, there was $13.9 million of total unrecognized compensation cost related to unvested stock options. As of December 31, 2014, the Company expects to recognize this cost over a remaining weighted-average period of 2.5 years.

8. Income taxes

As of December 31, 2014, the Company had federal net operating loss carryforwards of approximately $108.0 million and state net operating loss carryforwards of $106.9 million, which are available to reduce future taxable income. The Company also had federal tax credits of $2.3 million and state tax credits of $842,000, which may be used to offset future tax liabilities. The net operating loss (NOL) and tax credit carryforwards will expire at various dates through 2034. 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 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:

	Year ended December 31,	
	2013	2014
Income tax benefit using U.S. federal statutory rate	34.00%	34.00%
State tax benefit, net of federal benefit	4.69%	4.73%
Research and development tax credits	1.67%	1.51%
Permanent items	(4.76)%	(6.25)%
Change in the valuation allowance	(35.20)%	(34.34)%
Other	(0.40)%	0.35%
	—%	—%

Verastem, Inc.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

8. Income taxes (Continued)

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

	December 31,	
	2013	2014
Deferred tax assets:		
Net operating loss carryforwards	$ 25,782	$ 42,352
Capitalized research and development	1,839	2,154
Research and development credits	1,856	2,880
Stock-based compensation	511	726
Other	(4)	199
Gross deferred tax assets	29,984	48,311
Valuation allowance	(29,984)	(48,311)
Net deferred tax asset	$ —	$ —

The Company has recorded a valuation allowance against its deferred tax assets at December 31, 2014 and 2013 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 $10.3 million, $14.5 million and $18.3 million in the years ended December 31, 2012, 2013 and 2014, respectively, primarily relates to the net loss incurred by the Company.

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. From inception and through December 31, 2014, 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 consolidated 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.

9. Commitments and contingencies

In May 2011, the Company entered into a non-cancelable operating lease for office and laboratory space, which expired on October 31, 2014. The lease agreement provided for free rent for the first four months of the lease term and included escalating rent payments. Rent expense was recorded on a straight-line basis over the lease term, accordingly. The Company secured a letter of credit for $86,000 in connection with the lease, which was released upon expiration of the lease.

Verastem, Inc.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

9. Commitments and contingencies (Continued)

On April 15, 2014, the Company entered into a lease agreement for approximately 15,197 square feet of office and laboratory space in Needham, Massachusetts. The lease term commenced on April 15, 2014 and expires on September 30, 2019. The Company began using the leased premises as its corporate headquarters and commenced rent payments effective September 22, 2014. The Company has agreed to pay an initial annual base rent of approximately $493,000, which base rent increases after every twelve-month period during the lease term to approximately $554,000 for the last twelve-month period. The Company is recording rent expense on a straight-line basis, beginning in April 2014. The Company also received a tenant improvement allowance of approximately $684,000 in connection with the lease. The Company has accounted for the allowance as a lease incentive, which is being recorded as a reduction to rent expense over the lease term. Deferred rent and lease incentive obligation are included in accrued expenses (current portion) and other liabilities (noncurrent portion) in the consolidated balance sheet. The Company has also agreed to pay its proportionate share of increases in operating expenses and property taxes for the building in which the leased space is located. The Company has provided a security deposit in the form of a letter of credit in the amount of approximately $203,000, which may be reduced to approximately $162,000 on April 15, 2016. The amount is included in long term restricted cash on the consolidated balance sheet.

The minimum aggregate future lease commitments are as follows (in thousands):

2015	$ 497
2016	512
2017	527
2018	542
2019	415
Thereafter	—
Total	$ 2,493

The Company recorded rent expense of $323,000, $366,000 and $541,000 for the years ended December 31, 2012, 2013 and 2014, respectively.

Pursuant to the terms of various agreements, the Company may be required to pay various development, regulatory and commercial milestones. In addition, if any products related to these agreements are approved for sale, the Company may be required to pay significant royalties on future sales. The payment of these amounts, however, is contingent upon the occurrence of various future events, which have a high degree of uncertainty of occurring.

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 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

Verastem, Inc.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

10. License agreements (Continued)

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 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.

Under the license agreement with Poniard that the Company entered into in November 2011 relating to VS-4718 and certain other compounds, the Company paid an upfront license fee and agreed to pay Poniard milestone payments upon the achievement of specified development and regulatory milestones. In February 2014, the Company purchased the assets which were the subject of the license agreement with Poniard from Encarta, Inc. ("Encarta"), who had previously purchased these assets in 2013. In consideration for these assets, the Company issued 97,500 shares of common stock, a warrant to purchase 142,857 shares of common stock with an exercise price equal to $17.16 per share and paid $25,000. All existing obligations under the license agreement, including an achieved development milestone and an obligation to issue a warrant, were settled as part of this transaction. The Company incurred $1.2 million of research and development expense in 2014 as a result of this transaction. In connection with the asset purchase agreement, the Company also assumed the rights and obligations under the Scripps License Agreement. Pursuant to the Scripps License Agreement, the Company is obligated to pay Scripps potential product development milestone payments of up to an aggregate of $3,000,000 upon the achievement of specified development and regulatory milestones. In addition, the Company is obligated to pay Scripps low single-digit royalties as a percentage of net sales of licensed products, subject to adjustments in certain circumstances. The Company's obligation to pay royalties on net sales is on a country by country basis.

On May 11, 2012, the Company acquired from S*Bio Pte Ltd (S*Bio), compounds identified as dual inhibitors of PI3K and mTOR, including related patent rights. Under the agreement, the Company paid S*Bio an upfront fee of $350,000 and has agreed to pay S*Bio milestone payments of up to an aggregate of approximately $21.0 million upon the achievement of specified development and regulatory milestones. In addition, the Company agreed to pay to S*Bio tiered, low to mid single digit royalties as a percentage of annual net sales of each product containing an acquired compound as an ingredient. The obligation to pay royalties continues on a product by product and country by country basis until the expiration of all acquired patent rights covering the product in such country. If the Company obtains a license from a third party in order to commercialize an acquired compound contained in a product in a particular country, then the Company may deduct up to 50% of the amount paid to such third party from the royalty payments that Company owes to S*Bio for such product. The deduction is subject to specified limitations, including that in no event will any such

Verastem, Inc.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

10. License agreements (Continued)

deduction reduce a royalty payment owed to S*Bio by more than 50% as a result of all such deductions in the aggregate. There were no ongoing clinical trials at the time of the acquisition of the compounds, and the compounds acquired do not have alternative future uses, nor have they reached a stage of technological feasibility. As no processes or activities were acquired, the Company accounted for the transaction as an asset acquisition by recording the $350,000 payment made to S*Bio to research and development expense for the year ended December 31, 2012.

On July 11, 2012, the Company entered into a license agreement with Pfizer Inc. (Pfizer), under which Pfizer granted the Company worldwide, exclusive rights to research, develop, manufacture and commercialize products containing certain of Pfizer's inhibitors of focal adhesion kinase (Products) for all therapeutic, diagnostic and prophylactic uses in humans. The Company is solely responsible, at its expense, for the clinical development of the Products, which is to be conducted in accordance with an agreed-upon development plan. The Company is also responsible for all manufacturing and commercialization activities at its own expense. Pfizer is required to provide the Company with an initial quantity of clinical supply of one of the Products for an agreed upon price. Under the agreement, the Company made a one-time cash payment to Pfizer in the amount of $1.5 million and issued 192,012 shares of its common stock. Pfizer is also eligible to receive up to $2 million in developmental milestones and up to an additional $125 million based on the successful attainment of regulatory and commercial sales milestones. Pfizer is also eligible to receive high single to mid double digit royalties on future net sales of Products. The Company's royalty obligations with respect to each Product in each country begin on the date of first commercial sale of the Product in that country, and end on the later of 10 years after the date of first commercial sale of the Product in that country or the date of expiration or abandonment of the last claim contained in any issued patent or patent application licensed by Pfizer to the Company that covers the Product in that country. The Company accounted for the license agreement as the licensing of in-process research and development with no alternative future use. Accordingly, the Company recorded research and development expense for the year ended December 31, 2012 associated with the cash payment of $1.5 million and the fair value of shares of common stock issued to Pfizer of $2.0 million.

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 or post-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. The Company made contributions to the 401(k) Plan of $116,000, $160,000 and $219,000 for the years ended December 31, 2012, 2013 and 2014, respectively.

Verastem, Inc.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

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

	First Quarter Ended March 31, 2014	Second Quarter Ended June 30, 2014	Third Quarter Ended September 30, 2014	Fourth Quarter Ended December 31, 2014
Operating expenses:				
Research and development	$ 8,411	$ 8,305	$ 9,047	$ 9,685
General and administrative	4,723	4,782	4,341	4,313
Total operating expenses	13,134	13,087	13,388	13,998
Loss from operations	(13,134)	(13,087)	(13,388)	(13,998)
Interest income	72	65	56	49
Net loss	$ (13,062)	$ (13,022)	$ (13,332)	$ 13,949
Net loss per share—basic and diluted	$ (0.51)	$ (0.51)	$ (0.52)	$ (0.53)
Weighted-average number of common shares used in net loss per share—basic and diluted	25,478	25,669	25,811	26,248(a)

	First Quarter Ended March 31, 2013	Second Quarter Ended June 30, 2013	Third Quarter Ended September 30, 2013	Fourth Quarter Ended December 31, 2013
Operating expenses:				
Research and development	$ 5,296	$ 6,045	$ 6,789	$ 7,800
General and administrative	3,785	4,239	3,855	3,593
Total operating expenses	9,081	10,284	10,644	11,393
Loss from operations	(9,081)	(10,284)	(10,644)	(11,393)
Interest income	44	34	53	69
Net loss	$ (9,037)	$ (10,250)	$ (10,591)	$ (11,324)
Net loss per share—basic and diluted	$ (0.44)	$ (0.49)	$ (0.44)	$ (0.45)
Weighted-average number of common shares used in net loss per share—basic and diluted	20,483	20,729	24,127(b)	25,277

(a) In November and December 2014, the Company sold 1,346,676 shares of common stock under the Company's at-the-market equity offering program which resulted in net proceeds of $11.6 million.

(b) In July 2013, the Company closed a public offering in which it sold 4,255,000 shares of its common stock to the public at a price of $15.00 per share, including 555,000 shares issued pursuant to the exercise of the underwriters' option to purchase additional shares, which resulted in net proceeds of approximately $59.8 million.

13. Subsequent events

The Company reviews all activity subsequent to year end but prior to the issuance of the consolidated financial statements for events that could require disclosure or that could impact the

Verastem, Inc.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS (Continued)

13. Subsequent events (Continued)

carrying value of assets or liabilities as of the balance sheet date. Other than the events noted below, the Company is not aware of any subsequent events which would require recognition or disclosure in the financial statements.

In January 2015, the Company closed a public offering in which it sold 8,337,500 shares of its common stock to the public at a price of $6.50 per share, including 1,087,500 shares issued pursuant to the exercise of the underwriters' option to purchase additional shares. The offering was completed under the shelf registration statement that was filed on Form S-3 and declared effective by the Securities Exchange Commission on January 8, 2014. The net proceeds from this offering were approximately $50.9 million, after deducting underwriting discounts and commissions.

In January 2015, the Company sold an additional 276,663 shares of common stock under the at-the-market equity offering program with net proceeds of approximately $2.5 million, after deducting commissions and other offering expenses.

EXHIBIT INDEX

Exhibit number	Description of exhibit
3.1	Restated Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.1 to Annual Report on Form 10-K (File No. 001-35403) filed by the Registrant on March 30, 2012)
3.2	Amended and Restated 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)
4.1	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 the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on November 3, 2011)
4.2	Registration Rights Agreement, dated July 11, 2012, by and between the Registrant and Pfizer Inc. (incorporated by reference to Exhibit 4.1 to Current Report on Form 8-K (File No. 001-35403) filed by the Registrant on July 11, 2012)
4.3	Specimen certificate evidencing shares of common stock (incorporated by reference to Exhibit 4.1 to Amendment No. 3 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on January 13, 2012)
4.4	Common Stock Warrant Agreement between the Registrant and Encarta, Inc. dated February 21, 2014 (incorporated by reference to Exhibit 4.1 of the Quarterly Report on Form 10-Q (File No. 001-35403) filed by the Registrant on May 8, 2014)
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)

Exhibit number	Description of exhibit
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 April 15, 2014, between the Registrant and Intercontinental Fund III 117 Kendrick Street LLC (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K (File No. 001-35403, filed by the Registrant on April 18, 2014)
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 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 May 5, 2008 by and between The Scripps Research Institute and Poniard Pharmaceuticals, Inc. (Registrant assumed the rights and obligations of Encarta, Inc., which previously assumed the rights and obligations from Poniard Pharmaceuticals, Inc., on February 21, 2014) (incorporated by reference to Exhibit 10.1 of the Quarterly Report on Form 10-Q (File No. 001-35403) filed by the Registrant on May 8, 2014)
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 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)
10.15#	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)
10.16#	Restricted Stock Purchase Agreement, dated August 11, 2010, between the Registrant and Christoph Westphal (incorporated by reference to Exhibit 10.17 to Annual Report on Form 10-K (File No. 001-35403) filed by the Registrant on March 30, 2012)
10.17#	Employment Agreement, dated March 1, 2012, between the Registrant and Daniel Paterson (incorporated by reference to Exhibit 10.18 to Annual Report on Form 10-K (File No. 001-35403) filed by the Registrant on March 26, 2013)

Exhibit number	Description of exhibit
10.18†	Asset Purchase Agreement, dated May 10, 2012, by and between the Registrant and S*Rio Pte Ltd. (incorporated by reference to Exhibit 10.1 to Quarterly Report on Form 10-Q (File No. 001-35403) filed by the Registrant on August 13, 2012)
10.19†	License Agreement, dated July 11, 2012, by and between the Registrant and Pfizer Inc. (incorporated by reference to Exhibit 10.2 to Quarterly Report on Form 10-Q (File No. 001-35403) filed by the Registrant on August 13, 2012)
10.20#	Offer Letter, dated as of September 18, 2012, by and between the Registrant and Christoph Westphal, M.D., Ph.D. (incorporated by reference to Exhibit 10.1 to Quarterly Report on Form 10-Q (File No. 001-35403) filed by the Registrant on November 13, 2012)
10.21#	Restricted Stock Unit Agreement, dated as of September 18, 2012, by and between the Registrant and Christoph Westphal, M.D., Ph.D. (incorporated by reference to Exhibit 10.2 to Quarterly Report on Form 10-Q (File No. 001-35403) filed by the Registrant on November 13, 2012)
10.22#	Restricted Stock Unit Agreement, dated as of September 18, 2012, by and between the Registrant and Christoph Westphal, M.D., Ph.D. (incorporated by reference to Exhibit 10.3 to Quarterly Report on Form 10-Q (File No. 001-35403) filed by the Registrant on November 13, 2012)
10.23#	Employment Agreement, dated as of October 23, 2012, by and between the Registrant and Joanna Horobin (incorporated by reference to Exhibit 10.4 to Quarterly Report on Form 10-Q (File No. 001-35403) filed by the Registrant on November 13, 2012)
10.24#	Amendment to Form of Restricted Stock Unit Agreement under 2012 Incentive Plan (incorporated by reference to Exhibit 10.25 to Annual Report on Form 10-K (File No. 001-35403) filed by the Registrant on March 26, 2013)
10.25#	Employment Agreement, dated May 10, 2013, by and between the Registrant and John B. Green, CPA (incorporated by reference to Exhibit 99.2 to current report on Form 8-K (File No. 001-35403) filed by the Registrant on May 14, 2013)
10.26#	Letter Agreement, dated June 6, 2013, by and between the Registrant and Robert Forrester (incorporated by reference to Exhibit 10.2 to Quarterly Report on Form 10-Q (File No. 001-35403) filed by the Registrant on August 13, 2013)
10.27†	Letter Agreement, dated December 7, 2012, by and between the Registrant and Pfizer Inc.
10.28#	Amended and Restated Employment Agreement, dated November 22, 2013, by and between the Registrant and Robert Forrester
10.29#	Letter Agreement with Robert Forrester, dated June 6, 2013 (incorporated by reference to Exhibit 10.2 to Quarterly Report on Form 10-Q (File No. 001-35403) filed by the Registrant on August 13, 2013)
10.30#	Employment Agreement between the Registrant and Monica Singh (incorporated by reference to Exhibit 10.2 to the Quarterly Report on Form 10-Q (File No. 001-35403) filed by the Registrant on May 8, 2014.
21.1*	Subsidiaries of the Registrant
23.1*	Consent of Ernst & Young LLP
31.1*	Certification of the Chief Executive Officer pursuant to Exchange Act Rule 13a-14(a)

Exhibit number	Description of exhibit
31.2*	Certification of the Chief Financial Officer pursuant to Exchange Act Rule 13a-14(a)
32.1*	Certification of the 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 the Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS*	XBRL Instance Document
101.SCH*	XBRL Taxonomy Extension Schema Document
101.CAL*	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF*	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB*	XBRL Taxonomy Extension Label Linkbase Document

* Filed herewith.

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

‡ Confidential treatment requested under 17 C.F.R. §200.80(b)(4) and Rule 24b-2. The confidential portions of this exhibit have been omitted and are marked accordingly. The confidential portions have been provided separately to the SEC pursuant to the confidential treatment request.

Management contract or compensatory plan, contract or agreement.

Exhibit 21.1

List of Registrant's Subsidiaries

Verastem Securities Company, incorporated in Massachusetts, a wholly owned subsidiary.

QuickLinks

Exhibit 23.1

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the following Registration Statements:

(1) Registration Statement (Form S-3 No. 333-192968) of Verastem, Inc.,

(2) Registration Statement (Form S-8 No. 333-180475) pertaining to the 2010 Equity Incentive Plan and the 2012 Incentive Plan of Verastem, Inc.,

(3) Registration Statement (Form S-8 No. 333-190578) pertaining to the 2012 Incentive Plan of Verastem, Inc.,

(4) Registration Statement (Form S-8 No. 333-201075) pertaining to the 2014 Inducement Award Program of Verastem, Inc., and

(5) Registration Statement (Form S-8 No. 333-201076) pertaining to the 2012 Incentive Plan of Verastem, Inc.

of our report dated March 10, 2015 with respect to the consolidated financial statements of Verastem, Inc. included in this Annual Report (Form 10-K) of Verastem, Inc. for the year ended December 31, 2014.

/s/ Ernst & Young LLP

Boston, Massachusetts
March 10, 2015

QuickLinks

Exhibit 31.1

CERTIFICATIONS

I, Robert Forrester certify that:

1. I have reviewed this Annual Report on Form 10-K of Verastem, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

/s/ ROBERT FORRESTER

Robert Forrester
Chief Executive Officer

Date: March 10, 2015

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Exhibit 31.2

CERTIFICATIONS

I, John B. Green, CPA, certify that:

1. I have reviewed this Annual Report on Form 10-K of Verastem, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

/s/ JOHN B. GREEN, CPA

John B. Green, CPA
Chief Financial Officer

Date: March 10, 2015

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Exhibit 32.1

**CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the Annual Report on Form 10-K of Verastem, Inc. (the "Company") for the period ended December 31, 2014 as filed with the Securities and Exchange Commission (the "SEC") on the date hereof (the "Report"), the undersigned, Robert Forrester, Chief Executive Officer of the Company, hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to my knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 as amended; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

/s/ ROBERT FORRESTER

Robert Forrester
Chief Executive Officer

Date: March 10, 2015

A signed original of this written statement required by Section 906 has been provided to the Company and will be retained by the Company and furnished to the SEC or its staff upon request.

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Exhibit 32.2

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report on Form 10-K of Verastem, Inc. (the "Company") for the period ended December 31, 2014 as filed with the Securities and Exchange Commission (the "SEC") on the date hereof (the "Report"), the undersigned, John B. Green, CPA, Chief Financial Officer of the Company, hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to my knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 as amended; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

/s/ JOHN B. GREEN, CPA

John B. Green, CPA
Chief Financial Officer

Date: March 10, 2015

A signed original of this written statement required by Section 906 has been provided to the Company and will be retained by the Company and furnished to the SEC or its staff upon request.

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