

Verastem, Inc.  
Form 10-K  
March 13, 2018  
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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

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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, 2017

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	02494
(Address of principal executive offices)	(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	Emerging growth company
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If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

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, 2017 was \$79,779,254.

The number of shares outstanding of the registrant's common stock as of March 7, 2018 was 50,800,908.



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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 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 duvelisib and defactinib, and our PI3K and FAK 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, 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 and commercialization activities and fund operations for a specified period. The words “anticipate,” “believe,” “estimate,” “expect,” “intend,” “may,” “predict,” “project,” “target,” “potential,” “will,” “would,” “could,” “should,” “continue” and similar expressions are intended 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 we make. Applicable risks and uncertainties include the risks that the full data from the Phase 3 DUO™ study will not be consistent with the previously presented results of the study; that the preclinical testing of our product candidates and preliminary or interim data from clinical trials may not be predictive of the results or success of ongoing or later clinical trials; that data may not be available when expected, including for the Phase 3 DUO study; that even if data from clinical trials is positive, regulatory authorities may require additional studies for approval and the product may not prove to be safe and effective; that the degree of market acceptance of product candidates, if approved, may be lower than expected; that the timing, scope and rate of reimbursement for our product candidates is uncertain; that there may be competitive developments affecting our product candidates; that data may not be available when expected; that enrollment of clinical trials may take longer than expected; that our product candidates will cause unexpected safety events or result in an unmanageable safety profile as compared to their level of efficacy; that duvelisib will be ineffective at treating patients with lymphoid malignancies; that we will be unable to successfully initiate or complete the clinical development and eventual commercialization of our product candidates; that the development and commercialization of our product candidates will take longer or cost more than planned; that we may not have sufficient cash to fund our contemplated operations; that we or Infinity Pharmaceuticals, Inc. will fail to fully perform under the duvelisib license agreement; that we may be unable to make additional draws under our debt facility or obtain adequate financing in the future through product licensing, co-promotional arrangements, public or private equity, debt financing or otherwise; that we will not pursue or submit regulatory filings for our product candidates, including for duvelisib in patients with CLL/SLL or iNHL; acceptance or approval of our New Drug Application for duvelisib will not occur on the expected timeframe or at all and that our product candidates will not receive regulatory approval, become commercially successful products, or result in new treatment options being offered to patients. Other risks and uncertainties include those identified under the heading "Risk Factors" in this Annual Report on Form 10-K for the year ended December 31, 2017 and in any subsequent filings with the Securities and Exchange Commission (SEC).

As a result of these and other factors, we may not 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.



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PART I

Item 1. Business

OVERVIEW

We are a biopharmaceutical company focused on developing and commercializing drugs to improve the survival and quality of life of cancer patients. Our most advanced product candidates, duvelisib and defactinib, utilize a multi-faceted approach to treat cancers originating either in the blood or major organ systems. We are currently evaluating these compounds in both preclinical and clinical studies as potential therapies for certain cancers, including leukemia, lymphoma, lung cancer, ovarian cancer, mesothelioma, and pancreatic cancer. We believe that these compounds may be beneficial as therapeutics either as single agents or when used in combination with immuno-oncology agents or other current and emerging standard of care treatments in aggressive cancers that are poorly served by currently available therapies.

Duvelisib targets the Phosphoinositide 3-kinase (PI3K) signaling pathway. The PI3K signaling pathway plays a central role in cancer proliferation and survival. Duvelisib is an investigational oral therapy designed to attack both malignant B-cells and T-cells and disrupt the tumor microenvironment to help thwart their growth and proliferation through the dual inhibition of PI3K delta and gamma. Duvelisib is being developed for the treatment of patients with hematologic cancers including chronic lymphocytic leukemia and small lymphocytic lymphoma (CLL/SLL) and indolent non-Hodgkin lymphoma (iNHL), which includes follicular lymphoma (FL), and other subtypes of lymphoma, including peripheral T-cell lymphoma (PTCL). Duvelisib has U.S. Food and Drug Administration (FDA) Fast Track Designation for patients with CLL or PTCL who have received at least one prior therapy and for patients with FL who have received at least two prior therapies. In addition, duvelisib has orphan drug designation for patients with CLL/SLL and FL in the United States and European Union.

Duvelisib was evaluated in late- and mid-stage clinical trials, including DUO™, a randomized, Phase 3 monotherapy study in patients with relapsed or refractory CLL/SLL, and DYNAMO™, a single-arm, Phase 2 monotherapy study in patients with double-refractory iNHL, including FL, SLL, and marginal zone lymphoma (MZL). Both DUO and DYNAMO achieved their primary endpoints upon top-line analysis of efficacy data. We submitted a New Drug Application (NDA) to the FDA requesting the full approval of duvelisib for the treatment of patients with relapsed or refractory CLL/SLL and accelerated approval for the treatment of patients with relapsed or refractory FL in February 2018.

Defactinib is a targeted inhibitor of the Focal Adhesion Kinase (FAK) signaling pathway. FAK is a non-receptor tyrosine kinase encoded by the PTK-2 gene that is involved in cellular adhesion and, in cancer, metastatic capability. Similar to duvelisib, defactinib is also orally available and designed to be a potential therapy for patients to take at home under the advice of their physician. Defactinib has orphan drug designation in ovarian cancer in the United States and the European Union, and in mesothelioma in the United States, the European Union, and Australia.

Defactinib is currently being evaluated in a Phase 1b study in combination with Merck & Co.'s PD-1 inhibitor pembrolizumab and gemcitabine in patients with advanced pancreatic cancer, a Phase 1/2 clinical collaboration with Pfizer Inc. (Pfizer) and Merck KGaA to evaluate defactinib in combination with avelumab, an anti-PD-L1 antibody, in patients with ovarian cancer, and a Phase 1/2 study in collaboration with Cancer Research UK and Merck & Co. for the combination of defactinib with pembrolizumab in patients with non-small cell lung cancer (NSCLC),

mesothelioma or pancreatic cancer.

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## THE PROBLEM

Cancer is a group of diseases characterized by uncontrolled growth and spread of abnormal cells. The American Cancer Society estimates that in the United States in 2018, approximately 1.7 million new cases of cancer will be diagnosed and approximately 610,000 people will die from the disease. Current treatments for cancer include surgery, radiation therapy, chemotherapy, hormonal therapy, immunotherapy, and targeted therapy. Despite years of intensive research and clinical use, current treatments often fail to cure cancer. 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 following table sets forth the U.S. annual incidence of certain cancers, based on 2017 estimates from the National Cancer Institute's Surveillance, Epidemiology, and End Results Program (NCI; SEER).

Cancer type	U.S. annual incidence
Lymphoma	
Non-Hodgkin lymphoma	72,240
Chronic lymphocytic leukemia/small lymphocytic leukemia	20,110
Follicular lymphoma	14,448
Solid tumor	
Lung and bronchus cancer	222,500
Pancreatic cancer	53,670
Ovarian cancer	22,440

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 potential of oral, targeted therapies, along with the rapidly advancing field of immunotherapy, or using the body's immune system to fight cancer, are important new insights that present the opportunity to develop more effective cancer treatments.

## OUR STRATEGY

Our product candidates seek to utilize a multi-faceted approach to treat cancer by directly targeting the cancer cells, enhancing anti-tumor immunity, and modulating the local tumor microenvironment. Our goal is to build a leading biopharmaceutical company focused on the development and commercialization of novel drugs that use a multi-faceted approach to improving outcomes for patients with cancer.

Key elements of our strategy to achieve this goal are:

- Selectively build a commercial infrastructure in the U.S. for the potential launch of duvelisib in hematologic malignancies as an oral monotherapy for patients needing additional lines of therapy following previous treatment.
- Advance our product candidates through clinical development. We have ongoing clinical trials of duvelisib and defactinib both as single agents and in combination with other agents in several hematologic and solid tumor indications.
- Expand the indications in which our product candidates may be used. In parallel to CLL/SLL, iNHL, PTCL, NSCLC, ovarian cancer, pancreatic cancer and mesothelioma trials that we are currently conducting, we plan to pursue additional disease indications to expand the potential of our product candidates.



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- 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 agents. We may pursue the acquisition or in license of rights to additional agents from third parties that may supplement our internal programs and allow us to initiate clinical development of a diverse pipeline of agents more quickly.
- Build and maintain scientific leadership in the areas of lymphoid malignancies, immuno-oncology, and the tumor microenvironment. We plan to continue to conduct research in the hematological and immuno-oncology fields to further our understanding of the underlying biology of enhancing the body's immune response to tumors as well as 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 development of new therapies for the treatment of cancer.

## OUR PRODUCT CANDIDATES

We are focused on the development and commercialization of small molecules for optimized efficacy and safety primarily as orally available drug candidates. We have several product candidates currently in clinical trials, including duvelisib and defactinib. We are running clinical trials in cancers where there are limited treatment options, including CLL/SLL, iNHL, T-cell lymphoma, lung cancer, ovarian cancer, pancreatic cancer, mesothelioma, 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. As a result, the treatments may succeed at initially decreasing tumor burden but ultimately fail to kill all of the cancer cells or effectively disrupt the tumor microenvironment, potentially resulting in disease progression.

Our goal is to develop targeted agents that both specifically kill cancer cells and disrupt the tumor microenvironment to enhance the efficacy of cancer treatment. Agents that can modulate the tumor microenvironment to increase cytotoxic T-cell access to the tumor cells and decrease immunosuppressive T-cells in tumors have been sought after to increase the proportion of responding cancer patients and the duration of response (DOR) to cancer treatment.

### Chronic Lymphocytic Leukemia/Small Lymphocytic Leukemia, Non-Hodgkin Lymphoma

Hematologic malignancies are cancers of the blood or bone marrow such as CLL/SLL and non-Hodgkin lymphoma (NHL). In general, NHLs are a disease that occurs in patients over the age of 65.

The NCI estimates that there were 20,110 new cases of CLL/SLL in the U.S. in 2017 and that the five-year relative survival rate from 2007 to 2013 for patients with CLL/SLL was approximately 83%. As CLL/SLL is generally a slow-growing disease, the advent of new oral anti-cancer therapies since 2013 have been a significant advance as treatment options beyond chemotherapy or anti-B-lymphocyte antigen CD20 (CD20) immunotherapies, including ofatumumab. For example, the Bruton's Tyrosine Kinase (BTK) and B-cell lymphoma 2 (BCL-2) inhibitors have demonstrable activity in the treatment of CLL/SLL. However, evidence coming from studies on real-world use of these agents is revealing that a significant number of patients either relapse following treatment, become refractory to current agents, or are unable to tolerate treatment due to unmanageable side effects resulting from treatment, representing a significant medical need.



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The five year relative survival rate from 2007 to 2013 for patients with NHL was approximately 71%. The type and stage of the lymphoma can often provide useful information about a person's prognosis, but for some types of lymphomas the stage is less informative on its own. In these cases, other factors can give doctors a better idea about a person's prognosis. These factors are included in the International Prognostic Index and other metrics which take into account the patient's age, stage of disease, presence of metastases, performance status and blood levels of lactate dehydrogenase.

The potential of additional oral agents, particularly as a monotherapy that can be used in the general community physician's armamentarium, may hold significant value in the treatment of patients with CLL/SLL.

### Follicular Lymphoma

FL comprises 20% of all NHL and as many as 70% of the indolent lymphomas reported in American and European clinical trials. Common symptoms of FL include enlargement of the lymph nodes in the neck, underarms, abdomen, or groin, as well as fatigue, shortness of breath, night sweats, and weight loss. Often, patients with FL have no obvious symptoms of the disease at diagnosis. Most patients with FL are age 50 years and older and present with widespread disease at diagnosis. Nodal involvement is most common and is often accompanied by splenic and bone marrow disease. Rearrangement of the BCL-2 gene is present in more than 90% of patients with FL; overexpression of the BCL-2 protein is associated with the inability to eradicate the lymphoma by inhibiting apoptosis.

Despite the advanced stage, the median survival ranges from 8 to 15 years, leading to the designation of being indolent. Patients with advanced-stage FL are not cured with current therapeutic options. The rate of relapse is fairly consistent over time, even in patients who have achieved complete responses to treatment.

There are various treatment options for FL based on the severity of associated symptoms and the rate of cancer growth. If patients show no or very few symptoms, physicians may recommend not to treat the disease right away, an approach referred to as "active surveillance" (also known as "watchful waiting"). Active treatment is started if the patient begins to develop lymphoma-related symptoms or there are signs that the disease is progressing based on testing during follow-up visits.

FL is generally responsive to radiation and chemotherapy. Radiation alone can provide a long-lasting remission in some patients with limited disease. In more advanced stages, physicians may use one or more chemotherapy drugs or the monoclonal antibody rituximab (Rituxan), alone or in combination with other agents.

There have been only incremental advances in treatment options for FL beyond chemotherapy or immunotherapies like the antibodies against CD20, such as rituximab and obinutuzumab, and the overall clinical outlook for patients still remains poor. The potential of additional oral agents, particularly as a monotherapy that can be used in the general community physician's armamentarium, may hold significant value in the treatment of patients with FL.

### Peripheral T-Cell Lymphoma

PTCL consists of a group of rare and usually aggressive (fast-growing) NHLs that develop from mature T-cells. Most T-cell lymphomas are PTCLs, which collectively account for about 10% to 15% of all NHL cases in the United States.

PTCLs are sub-classified into various subtypes, each of which are typically considered to be separate diseases based on their distinct clinical differences. Most of these subtypes are very rare; the three most common subtypes of PTCL, peripheral T-cell lymphoma not otherwise specified (PTCL-NOS), anaplastic large-cell lymphoma (ALCL), and angioimmunoblastic T-cell lymphoma (AITL), account for approximately 70% of all PTCLs in the United States.

For most subtypes of PTCL, the frontline treatment regimen is typically a combination chemotherapy, such as CHOP (cyclophosphamide, doxorubicin, vincristine, prednisone), EPOCH (etoposide, vincristine, doxorubicin,

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cyclophosphamide, prednisone), or other multi-drug regimens. Because most patients with PTCL will relapse, some oncologists recommend giving high-dose chemotherapy followed by an autologous stem cell transplant (in which patients receive their own stem cells) to some patients who had a good response to their initial chemotherapy. While promising, there is no firm clinical data to support that undergoing a transplant in this setting is better than not undergoing a transplant.

The potential of additional oral agents, particularly as a monotherapy that can be used in the general community physician's armamentarium, may hold significant value in the treatment of patients with PTCL.

### Ovarian Cancer

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 NCI, 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 American Cancer Society estimates that in 2018 there will be approximately 22,200 new cases of ovarian cancer diagnosed and approximately 14,100 ovarian cancer related deaths.

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.

### Pancreatic Cancer

Pancreatic cancer is the tenth most common cancer diagnosed in the United States and the disease represents the third leading cause of cancer-related death in the country.

Pancreatic cancer often has a poor prognosis, even when diagnosed early. Pancreatic cancer typically spreads rapidly and is seldom detected in its early stages, which is a major reason why it is a leading cause of cancer death. Signs and symptoms may not appear until pancreatic cancer is so advanced that complete surgical removal is not possible. An estimated 54,000 Americans were diagnosed with pancreatic cancer in 2017 and over 43,000 were estimated to have died from the disease. Pancreatic cancer is one of the few cancers where survival has not improved significantly during the past 40 years. Pancreatic cancer has a very high mortality rate with approximately 92% of patients dying within five years of their initial diagnosis based on the five-year relative survival rate from 2007 to 2013. The median age for diagnosis is 70 with the disease affecting males slightly more than females.

Treatment options for pancreatic cancer are limited with surgical resection of the tumor possible in less than 20% of patients. Chemotherapy or chemotherapy plus radiation is offered to patients whose tumors are unable to be removed surgically. Immuno-oncology agents have not demonstrated a significant improvement in treatment outcome for patients with pancreatic cancer. The limited impact of chemotherapies and immunotherapies to improve the outcome may be due to the dense stroma that is prevalent in pancreatic tumors and the tumor microenvironment.

### Non-Small Cell Lung Cancer

According to the NCI, the most common types of 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 NCI estimates that in 2017 there were 222,500 new cases of lung cancer (both NSCLC and SCLC) in the United States and more than 150,000 deaths. Lung cancer is the leading cause of cancer related mortality in the United States. The five year relative survival rate from 2007 to 2013 for patients with lung cancer was approximately 18%.



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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.

### Mesothelioma

Mesothelioma is a form of cancer most often caused by asbestos, that affects the smooth lining of the chest, lungs, heart, and abdomen. The layer of tissue surrounding these organs is made up of mesothelial cells, hence the name mesothelioma. Mesothelioma most often forms in the pleural cavity of the chest or into the abdomen. Mesothelioma forms a solid tumor that begins as a result of insult to the tissues caused by asbestos particles, which penetrate into the pleural cavity of the chest.

Pleural mesothelioma accounts for approximately 2,500 - 3,000 cases a year in the United States. This disease affects the pleura, which is the thin balloon shaped lining of the lungs. In its early stages, mesothelioma is difficult to detect as it may start with a thickening of the pleural rind, or fluid, which can be associated with many other conditions. This rind is normally thin and smooth in the non-diseased state. In time it begins to demonstrate progression, forming a more pronounced irregular rind and nodules which coalesce into a crust that compresses and invades into adjacent structures compromising lung and cardiac function.

The symptoms of mesothelioma gradually become more noticeable, prompting the patient to seek a medical consultation. By this time the progression of the disease may already be too advanced, as the tumor may have spread to the lymph nodes and/or begun to metastasize to remote organs of the body like the brain, spleen, liver or kidneys.

### PI3K Inhibition Program

PI3K refers to a family of enzymes involved in multiple cellular functions, including cell proliferation and survival, cell differentiation, cell migration, and immunity. PI3K-delta and PI3K-gamma are two proteins with distinct and mostly non-overlapping roles believed to support the growth and survival of malignant B-cells and T-cells. Specifically, preclinical data suggest that PI3K-delta signaling can lead to the proliferation of malignant B-cells, and that both PI3K-gamma and PI3K-delta play an important role in the formation and maintenance of the supportive tumor microenvironment.

### Duvelisib

Our lead product candidate, duvelisib, is an oral, dual inhibitor of PI3K-delta and PI3K-gamma. Duvelisib is an investigational compound in clinical trials for hematologic malignancies, and its safety and efficacy have not yet been evaluated by the FDA or any other health authority for marketing authorization.

The clinical investigation program for duvelisib is supported by data from a Phase 1, open-label, dose-escalation study designed to evaluate the safety, pharmacokinetics and clinical activity of duvelisib in patients with advanced hematologic malignancies. The maximum tolerated dose of duvelisib was defined at 75 mg twice daily (BID) and the trial has been completed. A 25 mg BID dosing regimen was determined for further development based on efficacy, safety, pharmacokinetics and pharmacodynamics. Data from this study, presented in December 2014 at the Annual Meeting of the American Society for Hematology (ASH 2014), showed that duvelisib is clinically active in CLL/SLL, iNHL, and T-cell lymphoma, as well as other hematologic malignancies.

#### Chronic Lymphocytic Leukemia/Small Lymphocytic Leukemia, Non-Hodgkin Lymphoma

The FDA and European Medicines Agency (EMA) have granted orphan drug designation to duvelisib for the potential treatment of CLL/SLL, and the FDA has granted fast track designation to the investigation of duvelisib for the treatment of patients with CLL/SLL who have received at least one prior therapy. Duvelisib was evaluated for the treatment of CLL/SLL in the DUO™ study. The DUO study is a Phase 3, monotherapy, open-label, two- arm, randomized, superiority trial designed to evaluate the efficacy and safety of duvelisib at 25mg BID compared

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to ofatumumab, a monoclonal antibody treatment, administered to patients who have been diagnosed with CLL/SLL whose disease is relapsed or refractory. Patients in DUO that continue to derive benefit remain on treatment. DUO enrollment criteria included patients with CLL/SLL, whose disease had progressed during or relapsed after at least one previous CLL/SLL therapy. The primary endpoint of the study was Progression-Free Survival (PFS).

The investigation of duvelisib in DUO is supported by preliminary data from a Phase 1 study that demonstrated that duvelisib administered at 25 mg BID was clinically active in patients with relapsed or refractory CLL, with a 57% overall response rate (ORR) (17 of 30 evaluable patients), including one complete response, as per investigator assessment. At the time of the presentation of the study at ASH 2014, the median PFS in the 31 patients who received the 25 mg BID dose had not yet been reached with 66% of patients progression free at twelve months and 59% of patients progression free at 24 months.

CR: Complete Response; PR: Partial Response; PD: Progressive Disease; TP53mut/del(17p): high-risk cytogenetic markers

\*O'Brien et al., ASH 2014

The majority of side effects were Grade 1 or 2 in severity, reversible and/or clinically manageable. Across all doses evaluated in the study (n=55), the most common Grade 3 side effects were pneumonia (24%), neutropenia (18%) and anemia (16%). Grade 4 side effects included pneumonia in one patient (2%), neutropenia in 13 patients (24%) and anemia in one patient (2%).

The results from the DUO study were presented at the 2017 Annual Meeting of the American Society for Hematology conference (ASH 2017). The DUO study met its primary endpoint with oral duvelisib monotherapy achieving a statistically significant improvement in PFS compared to ofatumumab in patients with relapsed or refractory CLL/SLL per a blinded Independent Review Committee (IRC) using modified international workshop on CLL (iwCLL) or revised International Working Group (IWG) Response Criteria (median PFS=13.3 months versus 9.9 months, respectively; HR=0.52, p<0.0001), representing a 48% reduction in the risk of progression or death.

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Median PFS per IRC

\*Flinn et al., ASH 2017

Similar efficacy of duvelisib was observed regardless of whether patients had 17p deletion (del[17p]). The primary outcome of median PFS via IRC review in the del[17p] subpopulation significantly favored duvelisib over ofatumumab (median PFS=12.7 months versus 9.0 months, respectively; HR=0.41, p=0.0011), representing a 59% reduction in the risk of progression or death. Per investigator assessment, duvelisib demonstrated a median PFS of 17.6 months, compared to 9.7 months for ofatumumab (HR=0.40, p<0.0001). Duvelisib maintained a PFS advantage in all patient subgroups analyzed as a subset of pre-specified sensitivity analyses.

Median PFS per IRC for del[17p] Subpopulation

\*Flinn et al., ASH 2017

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Median PFS per Investigator Assessment

\*Flinn et al., ASH 2017

Median PFS by Subgroup

\*Flinn et al., ASH 2017

The secondary efficacy outcome of ORR via IRC assessment according to modified iwCLL/IWG criteria, significantly favored duvelisib over ofatumumab, 74% versus 45%, respectively ( $p < 0.0001$ ), and reduced lymph node burden by more than 50% in most patients compared to ofatumumab, 85% versus 16%, respectively. In the del[17p] subpopulation of patients, ORR was also significantly higher for duvelisib compared to ofatumumab, 70% versus 43%, respectively ( $p = 0.0182$ ).

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\*Flinn et al., ASH 2017

Patients who progressed in the DUO study were given the option to enroll in a crossover study to receive the opposite treatment. In the optional crossover study, 89 patients who were previously treated with ofatumumab in DUO and experienced confirmed disease progression were subsequently treated with duvelisib as a monotherapy. As in the parent DUO study, duvelisib demonstrated robust clinical activity in this crossover study with an ORR of 73%, a median DOR of 12.7 months and a median PFS of 15 months by investigator assessments.

In the DUO study, the overall survival in the intent to treat (ITT) population was similar for those randomized to duvelisib and to ofatumumab during the study (HR=0.99, p=0.4807), as expected there was no detrimental effect on overall survival. Though the FDA has noted that overall survival is the most reliable and therefore the preferred endpoint for approval of drugs for oncology indications in general, the FDA has publicly stated that it understands the challenges of showing an overall survival improvement in CLL/SLL, given the long natural history of the disease and availability of multiple therapies. Therefore, while they may request drug companies to collect overall survival data to ensure there is no detrimental effect on overall survival and to observe any potential improvement, an improvement in overall survival is not necessary for approval in CLL. Rather, improvements in PFS together with a favorable benefit-risk profile may be acceptable to receive FDA approval.

Following prolonged exposure, duvelisib, as a monotherapy, demonstrated a manageable safety profile, with results from this study consistent with the well-characterized safety profile of duvelisib monotherapy in patients with advanced hematologic malignancies in previous studies. For duvelisib-treated patients, the median time on treatment was 50.3 weeks (range, 0.9 - 160.0) compared to 23.1 weeks (range, 0.1 - 26.1) for ofatumumab. The most common Grade  $\geq 3$  treatment-emergent hematologic adverse events (occurring in more than 10% of patients) were neutropenia (30%) and anemia (13%). The most common Grade  $\geq 3$  non-hematologic treatment-emergent adverse events (occurring in more than 10% of patients) were diarrhea (15%), pneumonia (14%) and colitis (12%). The rate of severe opportunistic infections was 6%, including two patients (1%) with *Pneumocystis jirovecii* pneumonia (PJP), neither of whom was on prophylaxis for PJP at the time of the event. Adverse events led to discontinuation of treatment in 35% of patients. Approximately 40% of patients treated with duvelisib remained on treatment for over 18 months, with a median total follow-up of nearly two years.

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Adverse events of special interest infrequently led to discontinuation of duvelisib treatment (e.g., diarrhea (5%), colitis (5%), pneumonitis (2%), neutropenia (1%), pneumonia (1%), transaminase elevations (1%), and rash (1%). Duvelisib treatment-related adverse events leading to death (n=4) include general physical health deterioration (n=1), pneumonia staphylococcal (n=2) and sepsis (n=1)).

\*Flinn et al., ASH 2017

Indolent Non-Hodgkin Lymphoma

The FDA and EMA have granted orphan drug designation to duvelisib for the potential treatment of FL, and the FDA has granted Fast Track Designation to the investigation of duvelisib for the treatment of patients with FL who have received at least two prior therapies. The DYNAMO study is a Phase 2, open-label, single-arm monotherapy study evaluating the safety and efficacy of duvelisib dosed at 25 mg BID in 129 patients with iNHL. Patients in DYNAMO that continue to derive a benefit remain on treatment. DYNAMO enrollment criteria included patients with FL, the most common subtype of iNHL, MZL and SLL, whose disease is double-refractory to rituximab, an anti-CD20 monoclonal antibody, and to either chemotherapy or radioimmunotherapy and who must have progressed within six months of receiving their final dose of a previous therapy. The primary endpoint of the study was an ORR as assessed by IRC and according to the revised IWG Criteria, which includes a change in target nodal lesions in combination with other measurements to determine response to treatment.

The results from the DYNAMO study were presented at the 2016 Annual Meeting of the American Society for Hematology conference (ASH 2016). DYNAMO achieved the primary endpoint in a heavily pre-treated, double-refractory patient population with an ORR of 46% (p=0.0001) in the ITT population, as assessed by an IRC with a median DOR of 10 months. The breakdown of ORR in the three subtypes of iNHL for the overall study population was 41% in FL (n=83), 68% in SLL (n=28) and 33% in MZL (n=18). 83% of patients had a reduction of target nodal lesions in lymph nodes.

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\*Adapted from Flinn et al., ASH 2016

\*Flinn et al., ASH 2016

Duvelisib demonstrated a consistent and manageable safety profile with appropriate risk mitigation. The majority of adverse events were Grade 1 or 2 in severity, reversible and/or clinically manageable. The most common (greater than 5%) Grade 3 adverse effects were an increase in diarrhea (14%), anemia (10%), and neutropenia (9%). Grade 3 or 4 adverse effects of special interest included neutropenia (28%), infection (18%), diarrhea (15%), thrombocytopenia (13%), anemia (12%), pneumonia (9%), hepatotoxicity (8%), rash (7%), colitis (5%), and pneumonitis (2%). Serious opportunistic infections were less than 5% with none being fatal. Four treatment-related adverse events had the outcome of death (one septic shock; one viral infection; one drug reaction/eosinophilia/systemic symptoms; and one toxic epidermal necrolysis/sepsis syndrome).



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### T-cell Lymphoma, Aggressive NHL and Other Lymphomas

In the Phase 1 study, the ORR in patients with PTCL (n=16) was 50%, including three complete responses (CRs) and five partial responses (PRs). Responses were seen across the spectrum of PTCL subtypes, including CRs and PRs in patients with enteropathy-associated T-cell lymphoma (EATL), AITL, subcutaneous panniculitis-like T-cell lymphoma (SPTCL), and anaplastic large-cell lymphoma (ALCL), among others. DOR in the PTCL population ranged from 1.8 to 17.3 months with median PFS of 8.3 months and median overall survival of 8.4 months. In cutaneous T-cell lymphoma (CTCL) (n=19), the ORR was 32%, with six PRs. DOR ranged from 0.7 to 10.1 months and median PFS was 4.5 months. Median overall survival was not reached; however, the estimated probability of survival was determined to be of 90% at 6 months, 79% at 12 and 18 months, and 73% at 24 months. Duvelisib monotherapy demonstrated a manageable safety profile, with results from this study consistent with the well-characterized safety profile of duvelisib monotherapy in patients with hematologic malignancies in other studies. These clinical results were supported by preclinical findings showing that duvelisib exhibited cell-killing activity in vivo and promoted beneficial changes within the tumor microenvironment.

During 2017, the FDA granted Fast Track designation for the treatment of patients with PTCL, who have received at least one prior therapy. During the first quarter of 2018, we initiated an open-label, multicenter, Phase 2 clinical trial evaluating the efficacy and safety of duvelisib in patients with relapsed or refractory PTCL. We expect the study to be conducted in both the United States, the European Union, and Japan.

### FAK Inhibition Program

Our product candidates that inhibit FAK utilize a multi-faceted approach to treat cancer by enhancing anti-tumor immunity and modulating the local tumor microenvironment. Our lead FAK inhibitor is known as defactinib. The effects of FAK inhibition on the tumor microenvironment make defactinib a good candidate for combination therapy with immuno-oncology agents and other anti-cancer compounds. 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.

In September 2015, researchers from the University of Edinburgh published a study in the journal *Cell* that highlights the potential of FAK inhibition to enable the body's immune system to fight cancer. The paper discussed results from preclinical research showing that FAK enables cancer cells to evade attack by the immune system. This research showed that genetic knock down of FAK or oral dosing of mice with a FAK inhibitor decreases immunosuppressive cells called T-regulatory cells (Figure 1a) and increases cytotoxic T-cells (Figure 1b) in skin cancer tumors leading to a reduction in tumor burden (Figure 1c). This work has since been expanded into pancreatic cancer and colorectal cancer models in which FAK inhibition similarly extends survival of tumor-bearing mice through increasing cytotoxic T-cells in the tumor and decreasing T regulatory cells as published in *Nature Medicine* in August, 2016. Additionally, FAK inhibition was found to decrease other key immunosuppressive cell populations in tumors, known as myeloid-derived suppressor cells and M2 tumor-associated macrophages. Coincident with this immuno-modulation, FAK inhibition was shown to substantially increase survival of mice when combined with an anti-PD-1 immune checkpoint antibody. These results have indicated the potential promise of FAK inhibitors in combination with immune checkpoint inhibitors in the clinic.

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FIGURE 1

\*Adapted from: Serrels et al. Nuclear FAK controls chemokine transcription, Tregs, and evasion of anti-tumor immunity. Cell. 2015.

In the 2016 Nature Medicine paper, preclinical data were presented (Jiang, et al) demonstrating that FAK inhibition reduces stromal density and increases T-cell entry into tumors. In this study, it was discovered that treating mice bearing pancreatic cancer tumors with a FAK inhibitor reduces stromal density. This was measured as a decrease in the number (Figure 2a) and proliferation (Figure 2b) of tumor-associated fibroblasts, together with a decrease in collagen and other extracellular matrix proteins (Figure 2c) in the tumors. The paper's authors went on to show that this reduction in stromal density by FAK inhibition augments the effectiveness of the chemotherapeutic agent gemcitabine, and also allowed cytotoxic T-cells to enter the tumors (Figure 2d) to induce more durable survival of transgenic mice bearing pancreatic tumors (Figure 3). We believe these data provide strong rationale for the clinical evaluation of FAK inhibitors, including defactinib, in combination with a PD-1 or PD-L1 antibody in patients with pancreatic and other cancers. Based on this research, we have initiated clinical trials to assess the combination of defactinib with either avelumab (anti-PD-L1) or pembrolizumab (anti-PD-1) for the treatment of patients with ovarian cancer, pancreatic cancer, mesothelioma, or NSCLC.

FIGURE 2

\*Adapted from: Jiang et al. Targeting focal adhesion kinase renders pancreatic cancers responsive to checkpoint immunotherapy. Nature Medicine. 2016.

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FIGURE 3

Vehicle: Placebo control; Immuno: Gem +/- anti-PD-1 +/- anti-CTLA-4

\*Adapted from: Jiang et al. Targeting focal adhesion kinase renders pancreatic cancers responsive to checkpoint immunotherapy. Nature Medicine. 2016.

Defactinib

Defactinib is an orally available small molecule kinase inhibitor designed to inhibit FAK signaling. We are currently evaluating defactinib as a potential therapy for ovarian cancer, pancreatic cancer, mesothelioma, NSCLC, and other solid tumors. Defactinib has orphan drug designation in ovarian cancer in the United States and the European Union and in mesothelioma in the United States, the European Union, and Australia.

The clinical evaluation of defactinib is supported by a growing body of preclinical research suggesting that FAK inhibition, when combined with PD-1 inhibitors, increases the anti-tumor activity of these immunotherapeutic agents. As published in the journals Cell and Nature Medicine, FAK inhibition has been shown to increase cytotoxic (CD8+) T-cells in tumors, decrease T-cell exhaustion, decrease immunosuppressive cell populations, enhance T-cell killing of tumor cells, and create a generally more favorable tumor microenvironment, which may allow for enhanced efficacy of immuno-oncology therapeutics.

Pancreatic cancer, along with other tumors such as ovarian cancer and prostate cancer, are tumor types in which immunotherapeutics have achieved limited clinical benefit, possibly due to the dense desmoplastic stroma and the abundance of immunosuppressive cells. Preclinical research has demonstrated that high stromal density prevents anti-cancer agents and T-cells from entering pancreatic tumors thereby limiting efficacy. In preclinical research conducted by us and others, FAK inhibition was shown to reduce stromal density and allow cytotoxic T-cells to better penetrate the tumor and kill the cancer cells. Collectively, these data provide strong rationale for combining our FAK inhibitors with checkpoint inhibitors in the clinic for pancreatic and other solid tumors.

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Phase 1/2 study with Pfizer and Merck KGaA in combination with immunotherapy in ovarian cancer. In March 2016, we announced a new clinical collaboration with Pfizer and Merck KGaA to evaluate defactinib in combination with avelumab in patients with ovarian cancer. Avelumab is a human programmed death ligand 1 (PD-L1), blocking antibody that binds to the PD-L1 ligand expressed on tumor cells.

Phase 1/2 study with Cancer Research United Kingdom (CRUK) in combination with pembrolizumab. In September 2016, we announced a new clinical collaboration with CRUK and Merck & Co. to evaluate defactinib in combination with pembrolizumab, a PD-1 inhibitor, in patients with NSCLC, mesothelioma, or pancreatic cancer.

Phase 1/1b study in combination with immunotherapy in pancreatic cancer. Defactinib is in a dose escalation study in combination with Merck & Co.'s PD-1 inhibitor pembrolizumab and gemcitabine in patients with advanced pancreatic cancer. This Phase 1 clinical trial is anticipated to enroll approximately 50 patients and is being conducted at the Washington University School of Medicine's Division of Oncology under the direction of Andrea Wang-Gillam, M.D., Ph.D., Clinical Director of the Gastrointestinal Oncology Program. This trial is primarily designed to evaluate the safety of the combination regimen and may also provide a greater understanding of how FAK inhibition in combination with immunotherapies could improve outcomes for patients with pancreatic cancer.

## OUR MANAGEMENT TEAM AND SCIENTIFIC CO-FOUNDERS AND ADVISORS

Our experienced management team includes our President and Chief Executive Officer, Robert Forrester, Chief Strategy Officer, Steven Bloom, Chief Financial Officer, Julie Feder, Chief Medical Officer, Diep Le, M.D., Ph.D., Chief Commercial Officer, Joseph Lobjacki, and Chief Operating Officer, Daniel Paterson.

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. Bloom joined Verastem in March 2014 and recently took on the role of Chief Strategy Officer, focusing on Corporate and Business Development, Medical Affairs, Patient Advocacy and Corporate Communications. Prior to joining the company, Mr. Bloom was Senior Vice President at Ziopharm Oncology where for 6 years he led business development and the commercial planning initiatives for a late stage oncology asset. Before joining Ziopharm, Mr. Bloom was Vice President for the health informatics company Pharmetrics and spent the first 19 years of his career at Eli Lilly and Company in leadership roles in marketing, sales and corporate affairs.

Ms. Feder joined Verastem in July 2017 as our Chief Financial Officer. Ms. Feder served as the Chief Financial Officer for the Clinton Health Access Initiative, Inc. (CHAI) for the previous six years. Prior to joining CHAI, Ms. Feder spent three years at Genzyme Corporation, first as Vice President of Internal Audit and also as Finance Integration Leader. In these roles, she managed the day-to-day operations of Genzyme's global internal audit function, while leading the Genzyme Global Finance integration into Sanofi's organization following Sanofi's acquisition of Genzyme.

Dr. Le joined us in October 2017 as our Chief Medical Officer, is a trained medical oncologist, board certified in internal medicine and has 15 years of drug development experience across all phases in both solid and hematologic malignancies as well as IND and NDA submissions. Dr. Le joins Verastem from MedImmune (a subsidiary of AstraZeneca) where she served as Vice President, Immuno-Oncology Innovative Medicines and led the product development teams for multiple high-priority immuno-oncology assets. Prior to joining MedImmune, Dr. Le held roles of increasing responsibility at Novartis and at GlaxoSmithKline where she led the MEK inhibitor, trametinib (Mekinist™), from the first-in-human studies to FDA approval.

Mr. Lobacki joined Verastem in January 2018 as our Chief Commercial Officer. He most recently served as the Chief Operating Officer of Finch Therapeutics Group and previously as the Chief Commercial Officer and Executive Council Member of Medivation, where he was responsible for the strategy and execution of commercial operations including Xtandi, a treatment for advanced prostate cancer. Previously, Mr. Lobacki was Senior Vice President and Chief Commercial Officer of Micromet Inc., where he oversaw commercial activities including

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medical affairs and strategic marketing. Prior to joining Micromet, Mr. Lobacki was Senior Vice President and General Manager at Genzyme Corporation, where he managed the launch of Mozobil and Clolar/Evoltra in the US and EU.

Mr. Paterson has over 25 years of experience in management roles at healthcare and biotechnology companies, including as chief executive officer, Chief Operating Officer and Chief Business Officer, and specific expertise in oncology drug and diagnostic product development, business development, and launch planning. 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 Vice President of 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 cancer stem cells. 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.

## 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 U.S. Patent and Trademark Office to determine priority of invention.

## Patents

Our patent portfolio includes issued and pending applications worldwide. These patent applications fall into three categories: (1) PI3K inhibition program; (2) FAK inhibition program; and (3) other programs.

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### PI3K inhibition program

We are currently developing the PI3K inhibitor duvelisib.

We have exclusively licensed a portfolio of patent applications owned by Intellikine LLC and Infinity Pharmaceuticals, Inc. (Infinity), which are directed to PI3K inhibitor compounds and methods of their use, for example, in cancer. Certain patent families are related to duvelisib. These patent families include issued patents having claims covering duvelisib generically and specifically. Also included are issued patents covering certain polymorphs of duvelisib. Exemplary patents covering duvelisib, pharmaceutical compositions comprising duvelisib, methods of use, polymorphs, and methods of manufacture include US 8,193,182; US 8,785,456, and US 9,216,982. These U.S. patents have issued and will expire between 2029 and 2032. Related issued and pending worldwide patents and applications with claims to duvelisib, pharmaceutical compounds, methods of use, polymorphs, and methods of manufacture are pending in about 40 countries. Additional patent applications related to certain methods of use and combination therapies, as issued, would expire between 2029 and 2036.

### FAK inhibition program

We are currently developing the FAK inhibitor defactinib.

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 defactinib. This patent family includes issued patents having claims covering defactinib generically and specifically. For example, US 7,928,109 covers the composition of matter of defactinib specifically and US 8,247,411 covers the composition of matter of defactinib generically. Also included are issued and pending patent applications having claims directed to methods of treatment and methods of making defactinib. For example, US 8,440,822 covers methods of making defactinib. 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 three patent families covering defactinib. One family is directed to compositions (e.g., oral dosage forms) of defactinib 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. The other two families are directed to methods of using a FAK inhibitor in combination with another agent, such as defactinib in combination with a mitogen-activated protein kinase kinases (MEK) inhibitor for treating a patient or defactinib in combination with an immunotherapeutic agent. Any U.S. patents that will issue in these families will have a statutory expiration date in February of 2035 and June of 2036.

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 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.

### Patent Term

The base term of a U.S. 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 U.S. patent can be lengthened by patent term adjustment, which compensates the owner of the patent for administrative delays at the U.S. Patent and Trademark Office. In some cases,



the term of a U.S. 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

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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 FDA 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 regulatory 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

### Infinity Pharmaceuticals, Inc.

In November 2016, we entered into an amended and restated license agreement with Infinity, under which we acquired an exclusive worldwide license for the research, development, commercialization, and manufacture of products in oncology indications containing duvelisib. In connection with the license agreement, we assumed operational and financial responsibility for certain activities that were part of Infinity's duvelisib program, including the DUO study for patients with relapsed/refractory CLL/SLL, and Infinity assumed financial responsibility for the shutdown of certain other clinical studies up to a maximum of \$4.5 million. We are obligated to use diligent efforts to develop and commercialize a product in an oncology indication containing duvelisib. During the term of the license agreement, Infinity has agreed not to research, develop, manufacture or commercialize duvelisib in any other indication in humans or animals.

Pursuant to the terms of the license agreement, we are required to make the following payments to Infinity in cash or, at our election, in whole or in part, in shares of our common stock: (i) \$6.0 million upon the completion of the DUO study if the results of the study meet certain pre-specified criteria, which was paid in cash by us to Infinity in October 2017, and (ii) \$22.0 million upon the approval of an NDA in the United States or an application for marketing authorization with a regulatory authority outside of the United States for a product in an oncology indication containing duvelisib. For any portion of any of the foregoing payments that we elect to issue in shares of our common stock in lieu of cash, the number of shares of common stock to be issued will be determined by multiplying (1) 1.025 by (2) the number of shares of common stock equal to (a) the amount of the payment to be paid in shares of common stock divided by (b) the average closing price of a share of common stock as quoted on Nasdaq for a twenty-day period following the public announcement of the applicable milestone event. The shares of common stock will be issued as unregistered securities, and we will have an obligation to promptly file a registration statement with the SEC to register such shares for resale. Any issuance of shares will be subject to the satisfaction of closing conditions, including that all material authorizations, consents, approvals and the like necessary for such issuance shall have been obtained.

We are also obligated to pay Infinity royalties on worldwide net sales of any products in an oncology indication containing duvelisib ranging from the mid-single digits to the high single digits. The royalties will expire on a product-by-product and country-by-country basis until the latest to occur of (i) the last-to-expire patent right covering the applicable product in the applicable country, (ii) the last-to-expire patent right covering the manufacture of the

applicable product in the country of manufacture of such product, (iii) the expiration of non-patent regulatory exclusivity in such country and (iv) ten years following the first commercial sale of a product in a country, provided that if royalties on net sales for a product in the United States are payable solely on the basis of non-patent regulatory exclusivity, the applicable royalty on net sales for such product in the United States will be reduced by 50%. The royalties are also subject to reduction by 50% of certain third-party royalty payments or patent litigation damages or settlements which might be required to be paid by us if litigation were to arise, with any such reductions capped at 50% of the amounts otherwise payable during the applicable royalty payment period.

In addition to the foregoing, we are obligated to pay Infinity an additional royalty of 4% on worldwide net sales of any products in an oncology indication containing duvelisib to cover the reimbursement of research and development costs owed by Infinity to Mundipharma International Corporation Limited (MICL) and Purdue Pharmaceutical Products L.P. (Purdue). Once Infinity has fully reimbursed MICL and Purdue, the royalty obligations will be reduced to 1% of net sales in the United States. These trailing MICL royalties are payable until

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the later to occur of the last-to-expire of specified patent rights and the expiration of non-patent regulatory exclusivities in a country. Each of the above royalty rates is reduced by 50% on a product-by-product and country-by-country basis if the applicable royalty is payable solely on the basis of non-patent regulatory exclusivity. In addition, the trailing MICL royalties are subject to reduction by 50% of certain third-party royalty payments or patent litigation damages or settlements which might be required to be paid by us if litigation were to arise, with any such reductions capped at 50% of the amounts otherwise payable during the applicable royalty payment period.

### Pfizer Inc.

On July 11, 2012, we entered into a license agreement with Pfizer under which Pfizer granted us worldwide, exclusive rights to research, develop, manufacture and commercialize products containing certain of Pfizer's inhibitors of FAK, including defactinib, 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 \$2.0 million in developmental milestones and up to an additional \$125.0 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.

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.

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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.

Our competitors may commence and complete clinical testing of their product candidates, obtain regulatory approvals and begin commercialization of their products sooner than we may for our own product candidates. These competitive products may have superior safety or efficacy, or be manufactured less expensively, than our product candidates. If we are unable to compete effectively against these companies on the basis of safety, efficacy or cost, then we may not be able to commercialize our product candidates or achieve a competitive position in the market. This would adversely affect our business.

### PI3K inhibition program

We believe that the following companies, among others, have developed or are in the clinical stage of development of compounds targeting PI3K:

- Gilead Sciences, Inc. has received approval from the FDA of idelalisib for the treatment of patients with CLL, SLL, or FL, and which we believe is conducting a Phase 1b clinical trial of acalisib (GS-9820);
- Bayer AG has received approval from the FDA of copanlisib for the treatment of patients with relapsed FL;
- Novartis, which we believe is conducting a Phase 2 clinical trial of buparlisib;
- AstraZeneca, which we believe is conducting Phase 2 clinical trials of ACP 319;
- TG Therapeutics, Inc., which we believe is conducting multiple clinical trials of TGR-1202; and

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- Incyte Corporation, which we believe is conducting a Phase 2 clinical trial of INCB-050465, and which we also believe is conducting a Phase 2 clinical trial of INCB-040093.

In addition, many companies are developing product candidates directed to disease targets such as Bruton's Tyrosine Kinase (BTK), B-cell lymphoma 2 (BCL-2), Janus Kinase (JAK), B-lymphocyte antigen CD-19, and programmed death 1/ligand 1 (PD-1/PD-L1), Cluster of Differentiation 79B antibody-drug conjugate (CD79B ADC), and pleiotropic pathways in the fields of hematology-oncology, including in the specific diseases for which we are currently developing duvelisib, or for which we may develop duvelisib or other PI3K inhibitors in the future. Such companies include:

- Pharmacyclics LLC, a wholly-owned subsidiary of AbbVie, through its collaboration with Janssen Biotech, which has received approval from the FDA of ibrutinib, a BTK inhibitor, for the treatment of patients with mantle cell lymphoma (MCL), CLL, MZL, SLL, or Waldenström's macroglobulinemia, and is conducting multiple late stage clinical studies of ibrutinib in additional hematologic malignancies;
- AbbVie, through its collaboration with Roche, which has received approval from the FDA of venetoclax, a BCL-2 inhibitor, for the treatment of patients with CLL, and is conducting multiple late stage clinical studies of venetoclax in additional hematologic malignancies;
- Celgene Corporation, which has received FDA approval of lenalidomide, an immunomodulator, for the treatment of patients with multiple myeloma, MCL, and myelodysplastic syndromes, and is conducting late stage clinical studies of lenalidomide in additional hematologic malignancies; we also believe that Celgene is conducting a Phase 1 clinical trial of CC-292, a BTK inhibitor, in patients with CLL;
- AstraZeneca, which we believe is conducting a Phase 3 clinical trial of ACP-196, a BTK inhibitor, in patients with CLL; and
- Incyte Corporation, which has received FDA approval of ruxolitinib, a JAK inhibitor, in patients with intermediate or high-risk myelofibrosis, and which we believe is conducting Phase 2 clinical trials in CLL.

## FAK inhibition program

There are other companies working to develop therapies to treat cancer including some who also target the tumor microenvironment. 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.

## 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 and drug product for our product candidates from third party manufacturers. We obtain our supplies from these manufacturers on a purchase order basis and do not have long term supply arrangements in place. We do not currently have arrangements in place for redundant supply or a second source for bulk drug substance and drug product. 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.

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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 (FDCA) 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 (GLP) regulations;
- submission to the FDA of an investigational new drug (IND) application, which must become effective before human clinical trials may begin;
- approval by an independent institutional review board (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 (GCP) to establish the safety and efficacy of the proposed drug for each indication;
- submission to the FDA of an 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 (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,

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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 scheduled to exceed \$2.4 million, and the sponsor of an approved NDA is also subject to annual program fees, based on the number of approved products. These fees are typically adjusted annually. User fee statutory authority expires every five years. The Prescription Drug User Fee Act, was re-authorized for an additional five years in 2017 until 2022. Fee waivers are available in certain circumstances, including a waiver of



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the application fee for an orphan drug application.

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

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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 (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.

### 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 (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



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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 (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

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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.

### 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 (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.

### Other regulatory requirements

Any drug manufactured or distributed by us pursuant to FDA approvals would be 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.

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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

Although we currently have no products approved for commercial sale, we may be subject to various federal and state laws pertaining to healthcare “fraud and abuse,” including anti-kickback laws and false claims laws, for activities related to future sales of any of our product candidates that may in the future receive regulatory and marketing approval. Anti-kickback laws generally prohibit a pharmaceutical manufacturer from soliciting, offering, receiving, or paying any remuneration to generate business, including the purchase, prescription or use of a particular drug. Although the specific provisions of these laws vary, their scope is generally broad and there may not be regulations, guidance or court decisions that apply the laws to particular industry practices. There is therefore a possibility that our practices might be challenged under such anti-kickback laws. False claims laws prohibit anyone from knowingly and willingly presenting, or causing to be presented, any claims for payment for reimbursed drugs or services to third party payors (including Medicare and Medicaid) that are false or fraudulent.

Laws and regulations have been enacted by the federal government and various states to regulate the sales and marketing practices of pharmaceutical manufacturers with marketed products. The laws and regulations generally limit financial interactions between manufacturers and healthcare providers and/or require disclosure to the government and public of such interactions. Many of these laws and regulations contain ambiguous requirements or require administrative guidance for implementation. Given the lack of clarity in laws and their implementation, any future activities (if we obtain approval and/or reimbursement from federal healthcare programs for our product candidates) could be subject to challenge.

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If our operations are found to be in violation of the fraud and abuse laws described above, or any other laws that apply to us, we may be subject to penalties, including, without limitation, civil, criminal, and administrative penalties, damages, monetary fines, disgorgement, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, and curtailment or restructuring of our operations.

### 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 (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.

### 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. We may also need to provide discounts to purchasers, private health plans or government healthcare programs. 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 healthcare costs, including price

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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 healthcare 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. 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.

### New legislation and regulations

From time to time, legislation is drafted, introduced and passed in the United States Congress that could significantly change the statutory provisions governing the testing, approval, manufacturing and marketing of pharmaceutical products. For example, in December 2016, Congress enacted and President Obama signed into law the 21st Century Cures Act, that amends a number of sections of the FDCA, including provisions related to medical device approval. 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.

In the United States, federal and state governments continue to propose and pass legislation designed to reform delivery of, or payment for, healthcare, which include initiatives to reduce the cost of healthcare. For example, in March 2010, the United States Congress enacted the Patient Protection and Affordable Care Act and the Health Care and Education Reconciliation Act, or the Health Care Reform Act, which expanded healthcare coverage through Medicaid expansion and the implementation of the individual mandate for health insurance coverage and which included changes to the coverage and reimbursement of drug products under government healthcare programs as well as the imposition of annual fees on manufacturers of branded pharmaceuticals. Under the Trump administration, there have been ongoing efforts to modify or repeal all or certain provisions of the Health Care Reform Act. The Trump administration may also take executive action in the absence of legislative action. For example, in October 2017, the President announced that his administration will withhold the cost-sharing subsidies paid to health insurance exchange plans serving low-income enrollees. Actions by the administration are widely expected to lead to fewer Americans having more comprehensive health insurance compliant with the Health Care Reform Act, even in the absence of a



legislative repeal. Tax reform legislation was also enacted at the end of 2017 that includes provisions that will affect healthcare insurance coverage and payment, such as the elimination of the tax penalty for individuals who do not maintain sufficient health insurance coverage beginning in 2019 (the so-called “individual mandate”). In a November 2017 report, the Congressional Budget Office estimates that the elimination will increase the number of uninsured by 4 million in 2019 and 13 million in 2027.

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There have also been efforts by government officials or legislators to implement measures to regulate prices or payment for pharmaceutical products, including legislation on drug importation. Recently, there has been considerable public and government scrutiny of pharmaceutical pricing and proposals to address the perceived high cost of pharmaceuticals. There have also been recent state legislative efforts to address drug costs, which generally have focused on increasing transparency around drug costs or limiting drug prices.

Adoption of new legislation at the federal or state level could affect demand for, or pricing of, our product candidates if approved for sale. We cannot predict the ultimate content, timing or effect of any changes to the Health Care Reform Act or other federal and state reform efforts. There is no assurance that federal or state healthcare reform will not adversely affect our future business and financial results.

**EMPLOYEES**

As of February 28, 2018, we had 69 full time equivalent 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 28, 2018.

Name	Age	Position
Robert Forrester	54	President, Chief Executive Officer
Steven Bloom	57	Chief Strategy Officer
Julie B. Feder	47	Chief Financial Officer
Diep Le, M.D., Ph.D.	50	Chief Medical Officer
Joseph Lobacki	59	Chief Commercial Officer
Daniel Paterson	57	Chief Operating Officer

Robert Forrester 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.

Steven Bloom has served as our Chief Strategy Officer since December 2017, our Senior Vice President of Corporate Development from January 2017 to November 2017 and as our Vice President of Commercial Planning and External Affairs from January 2015 until January 2017. Prior to joining us in March 2014, Mr. Bloom served as Senior Vice President at Ziopharm Oncology from March 2008 to March 2014. Before joining Ziopharm, Mr. Bloom was Vice President for the health informatics company Pharmedics and spent the first 19 years of his career at Eli Lilly and

Company in leadership roles in marketing, sales and corporate affairs.

Julie B. Feder has served as our Chief Financial Officer since July 2017. Prior to joining us, Ms. Feder served as the Chief Financial Officer for the Clinton Health Access Initiative (CHAI) from September 2011 to July 2017. Prior to joining CHAI, Ms. Feder spent three years at Genzyme Corporation, first as Vice President of Internal Audit and also as Finance Integration Leader. In these roles, she managed the day-to-day operations of Genzyme's global internal audit function, while leading the Genzyme Global Finance integration into Sanofi's organization following Sanofi's acquisition of Genzyme.

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Diep Le, M.D., Ph.D. has served as our Chief Medical Officer since October 2017. Prior to joining us, Dr. Le served as the Vice President, Immuno-Oncology Innovative Medicines at MedImmune (a subsidiary of AstraZeneca) from October 2015 to June 2017 and led the product development teams for multiple high-priority immuno-oncology assets. Prior to that, Dr. Le served as the Executive Director and Global Clinical Program Lead at Novartis Oncology from October 2013 to October 2015, and various roles of increasing responsibility at GlaxoSmithKline from June 2009 to October 2013, where she led the MEK inhibitor, trametinib (Mekinist™), from the first-in-human studies to FDA approval.

Joseph Lobacki has served as our Chief Commercial Officer since January 2018. Prior to joining us, Mr. Lobacki served as the Chief Operating Officer of Finch Therapeutics Group from November 2016 to December 2017, the Chief Commercial Officer and Executive Council Member of Medivation, Inc. from December 2014 to October 2016, and as the General Manager of Oncology at Idera Pharmaceuticals from April 2014 to December 2014. Prior to that Mr. Lobacki served as a commercial and business operations consultant for biotechnology companies from June 2012 to April 2014 and as the Senior Vice President and Chief Commercial Officer of Micromet Inc., where he oversaw commercial activities including medical affairs and strategic marketing.

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 in March 2012, Mr. Paterson was a consultant in 2011. From 2009 through 2010, Mr. Paterson was the Chief Operating Officer of On Q ity. Mr. Paterson was the President and Chief Executive Officer 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.

## 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](http://www.verastem.com). We make available, free of charge on our website, our annual reports 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 (the Exchange Act) as soon as reasonably practicable after we electronically file those reports with, or furnish them to, the SEC. We also make available, free of charge on our website, the reports filed with the SEC by our executive officers, directors and 10% stockholders pursuant to Section 16 under the Exchange Act as soon as reasonably practicable after copies of those filings are provided to us by those persons. The information contained on, or that can be accessed through, our website is not a part of or incorporated by reference in this Annual Report on Form 10 K.

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ITEM 1A. Risk Factors.

RISKS RELATED TO THE DEVELOPMENT AND COMMERCIALIZATION OF OUR PRODUCT CANDIDATES

Preclinical testing and clinical trials of our product candidates may not be successful. In the near term, we are dependent on the success of our PI3K inhibitor program. If our New Drug Application (NDA) for duvelisib is not accepted by the U.S. Food and Drug Administration (FDA), we are unable to obtain marketing approval for or successfully commercialize duvelisib, or any of our other product candidates, or if we 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 product candidates, including duvelisib, for which we are conducting clinical trials in multiple indications and submitted an NDA to the FDA requesting approval in February 2018. Our ability to generate product revenues will depend heavily on the successful development and potential commercialization of our product candidates. The success of our product candidates will depend on several factors, including the following:

- initiation and successful enrollment and completion of our clinical trials
- receipt of marketing approvals from the FDA and other regulatory authorities for our product candidates, including pricing approvals where required, as well as securing acceptance and approval of the NDA we submitted for duvelisib
- 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
  - securing and maintaining coverage and adequate reimbursement for our products from third party payors;
- effectively competing with other therapies and
- a continued acceptable safety and efficacy profile of the products following approval.

Many of these factors are beyond our control, including clinical development, the regulatory submission process, potential threats to our intellectual property rights and the manufacturing, marketing and sales efforts of any collaborator. 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.

Even if duvelisib, or any of our other product candidates, receives marketing approval, it 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 duvelisib, or any of our other product candidates, receives marketing approval, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, healthcare payors and others in the medical community. If duvelisib does not achieve an adequate level of acceptance, or if we are unable to increase market acceptance of duvelisib as compared to existing or competitive products, we may not generate significant product revenues and we may not become profitable. In addition, clinical studies of duvelisib showed side effects that may need to be managed to be profitable. The degree of market acceptance of duvelisib, if approved for commercial sale, will depend on a number of factors, including:

- efficacy and potential advantages compared to alternative treatments;
- convenience and ease of administration compared to alternative treatments;

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- the ability to offer duvelisib for sale at competitive prices;
- the willingness of the target patient population to try new therapies and of physicians to prescribe duvelisib;
- the line of therapy duvelisib is designated under physician treatment guidelines;
- changes in the standard of care for the targeted indications for duvelisib;
- limitations or warnings, including distribution or use restrictions, contained in the approved labeling for duvelisib;
- the strength of marketing and distribution support;
- sufficient third-party coverage and reimbursement;
- the ability of the medical community to appropriately recognize and manage side effects;
- safety concerns with similar products marketed by others; and
- the prevalence and severity of any side effects as a result of treatment with duvelisib.

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, a further review and analysis of this data may change the conclusions drawn from this unaudited data indicating less promising results than we currently anticipate.

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. There also may be significant variability in the safety results obtained through the long-term follow-up of patients from ongoing studies. We do not know whether any clinical trial we may conduct or follow-up data we collect will demonstrate consistent or adequate efficacy and/or 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. The FDA or other regulatory authorities may require additional testing to substantiate our claims from our Phase 3 DUO, Phase 2 DYNAMO and other studies, which could delay or prevent marketing approval for duvelisib.

A failure of one or more clinical trials could indicate a higher likelihood that subsequent clinical trials of the same product candidate in the same or other indications or subsequent clinical trials of other related product candidates will be unsuccessful for the same reasons as the unsuccessful clinical trials.

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,

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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 including imposition of a Risk Evaluation and Mitigation Strategy (REMS), 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.

The FDA and foreign regulatory authorities may determine that the results from our ongoing and future trials 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 our product candidates could increase materially and their 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. 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 FDA or similar regulatory authorities outside the United States. In addition, there are a number of ongoing clinical trials being conducted by other companies 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:

- the size and nature of the patient population
- severity of the disease under investigation

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- eligibility criteria for the study in question
- perceived risks and benefits of the product candidate under study in relation to other available treatments including any new treatments that may be approved for the indications we are investigating
- 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.

Furthermore, enrolled patients may drop out of a clinical trial, which could impair the validity or statistical significance of the clinical trial. A number of factors can influence the patient discontinuation rate, including, but not limited to:

- the inclusion of a placebo arm in a trial;
- possible inactivity or low activity of the product candidate being tested at one or more of the dose levels being tested;
- the occurrence of adverse side effects, whether or not related to the product candidate; and
- the availability of numerous alternative treatment options, including clinical trials evaluating competing product candidates, that may induce patients to discontinue their participation in the trial.

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. Patients in our clinical trials have experienced serious adverse events, deemed by us and the clinical investigator to be related to our product candidates. Serious adverse events generally refer to adverse events, 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 outcomes.

Defactinib is in our Phase 1 and Phase 2 clinical trials and the development program continues to progress. The toxicities reported thus far are consistent with other drugs in this class.

As a result of adverse events observed to date, 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.



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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.

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 could differ in significant ways from earlier stage clinical trials, which could cause the outcome of the 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, including us, have suffered significant setbacks in late stage clinical trials after achieving positive results in an earlier stage of development. 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.

Our approach to the treatment of cancer through the killing of cancer cells and disruption of the tumor microenvironment is unproven, and we do not know whether we will be able to develop any products of commercial value.

We are developing and commercializing product candidates to treat cancer by using targeted agents to kill cancer cells or disrupt the tumor microenvironment and thereby thwart their growth and proliferation of cancer cells. Research on the use of small molecules to inhibit PI3K and FAK signaling pathways and disrupt the tumor microenvironment is an emerging field and, consequently, there is uncertainty about whether duvelisib and defactinib are effective in improving outcomes for patients with cancer. With respect to our FAK inhibition program, there is some debate in the scientific community regarding cancer stem cells (CSCs), the existence of these cells, the defining characteristics of these cells, as well as whether targeting such cells is an effective approach to treating cancer. Some believe that targeting CSCs as part of our multi-faceted approach should be sufficient for a positive clinical outcome, while others believe that, at times or always, the use of FAK inhibitors that reduce CSCs should be coupled with conventional chemotherapies for a positive clinical outcome.

Any products that we develop may not effectively target cancer cells, enhance anti-tumor immunity, or modulate the local tumor microenvironment. While we are currently conducting clinical trials for product candidates that we believe will attack cancer cells through the inhibition of the PI3K or FAK signaling pathways and potentially disrupt the tumor microenvironment, we may not ultimately be successful in demonstrating their efficacy, alone or in combination with other treatments.

The approval of our product candidates as part of a combination therapy for the treatment of certain cancers may be more costly than our prior clinical trials, may take longer to achieve regulatory approval, may be associated with new,

more severe or serious and unanticipated adverse events, and may have a smaller market opportunity.

Part of our current business model involves conducting clinical trials to study the effects of combining our product candidates with other approved and investigational targeted therapies, chemotherapies, and immunotherapies to treat patients with cancer. Regulatory approval for a combination treatment generally requires clinical trials to evaluate the activity of each component of the combination treatment. As a result, it may be more difficult and costly to obtain regulatory approval of our product candidate for use as part of a combination treatment than obtaining regulatory approval of our product candidates alone. In addition, we also risk losing the supply of any approved or investigational product being combined with our product candidate in these clinical trials. Furthermore, the potential market opportunity for our product candidates is difficult to estimate precisely. For instance, if one of

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our product candidates receives regulatory approval from a combination study, it may be approved solely for use in combination with the approved or investigational product in a particular indication and the market opportunity our product candidate would be dependent upon the continued use and availability of the approved or investigational product. In addition, because physicians, patients and third-party payors may be sensitive to the addition of the cost of our product candidates to the cost of treatment with the other products, we may experience downward pressure on the price that we can charge for our product candidates if they receive regulatory approval. Further, we cannot be sure that physicians will view our product candidates, if approved as part of a combination treatment, as sufficiently superior to a treatment regimen consisting of only the approved or investigational product. Additionally, the adverse side effects of our product candidates may be enhanced when combined with other products. If such adverse side effects are experienced, we could be required to conduct additional pre-clinical and clinical studies and if such adverse side effects are severe, we may not be able to continue the clinical trials of the combination therapy because the risks may outweigh the therapeutic benefit of the combination.

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

We may seek to acquire new compounds and product candidates from other pharmaceutical and biotechnology companies, academic scientists and other researchers, such as our exclusive in-license from Infinity Pharmaceuticals, Inc. (Infinity) to research, develop, commercialize, and manufacture products in oncology indications containing duvelisib. The success of this strategy depends partly upon our ability to identify, select, discover and acquire promising pharmaceutical product candidates and products. The process of proposing, negotiating and implementing a license or acquisition of a product candidate or approved product is lengthy and complex. Other companies, including some with substantially greater financial, marketing and sales resources, may compete with us for the license or acquisition of product candidates and approved products. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We have limited resources to identify and execute the acquisition or in-licensing of third-party products, businesses and technologies and integrate them into our current infrastructure. Moreover, we may devote resources to potential acquisitions or in-licensing opportunities that are never completed, or we may fail to realize the anticipated benefits of such efforts. 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. Any product candidate that we acquire may require additional development efforts prior to commercial sale, including manufacturing, pre-clinical testing, extensive clinical testing and approval by the FDA and applicable foreign regulatory authorities. All product candidates are prone to risks of failure typical of pharmaceutical product development.

In addition, future product or business acquisitions may entail numerous operational and financial risks, including:

- exposure to unknown liabilities
- disruption of our business and diversion of our management's time and attention to develop acquired products, product candidates or technologies
- higher than expected acquisition and integration costs
- increased amortization expenses and
- incurrence of substantial debt, dilutive issuances of securities or depletion of cash to pay for acquisitions.
- Future business acquisitions may also entail certain additional risks, such as:
- difficulty in combining the operations and personnel of any acquired businesses with our operations and personnel
- impairment of relationships with key suppliers or customers of any acquired businesses due to changes in management and ownership and
- inability to motivate key employees of any acquired businesses.

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. Marketing our



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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, a drug 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. Failure to obtain regulatory approval in one country may have a negative effect on the regulatory approval process in others. 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.

We have limited experience in marketing and commercializing product candidates. 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 limited 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, we will face significant increased costs as we undertake commercialization activities for any of our product candidates, including duvelisib, and 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 and building out a commercialization operation generally.

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

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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 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, including Gilead Sciences, Inc., Abbvie, Pharmacyclics LLC, Roche, Celgene Corporation, AstraZeneca, Incyte Corporation, TG Therapeutics, Inc., Novartis and others. 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 any of our product candidates are approved, they will be priced at a significant premium over competitive generic products.

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 or third-party coverage and reimbursement policies, which would harm our business.

The regulations that govern marketing approvals, pricing and reimbursement for new drug products vary widely from country to country. 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 coverage and adequate 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

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will cover 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 coverage and reimbursement will be available for any product that we commercialize and, if reimbursement is available, the level of reimbursement. Coverage and reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. Obtaining coverage and 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 coverage and reimbursement are not available or reimbursement 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 coverage and 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

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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 LICENSE AGREEMENT WITH INFINITY

If we do not realize the anticipated benefits of our license agreement with Infinity for the duvelisib program, our business could be adversely affected.

Our license agreement with Infinity for the duvelisib program may fail to further our business strategy as anticipated or to achieve anticipated benefits and success. We may make or have made assumptions relating to the impact of the acquisition of the duvelisib program on our financial results relating to numerous matters, including:

- transaction and integration costs;
- the cost of development and commercialization of duvelisib products; and
- other financial and strategic risks related to the license agreement with Infinity.

Further, we may incur higher than expected operating and transaction costs, and we may encounter general economic and business conditions that adversely affect us relating to our license agreement with Infinity. If one or more of these assumptions are incorrect, it could have an adverse effect on our business and operating results, and the benefits from our license agreement with Infinity for the duvelisib program may not be realized or be of the magnitude expected.

### RISKS RELATED TO OUR FINANCIAL POSITION AND NEED FOR ADDITIONAL CAPITAL

We require additional financing to execute our operating plan and continue to operate as a going concern.

Our audited consolidated financial statements for the year ended December 31, 2017 have been prepared assuming we will continue to operate as a going concern, but we believe that our cash, cash equivalents and investments at December 31, 2017 of \$86.7 million combined with our continuing operating losses raise substantial doubt about our ability to continue as such. Because we continue to experience net operating losses, our ability to continue as a going concern is subject to our ability to obtain necessary capital from outside sources, including obtaining additional capital from the sale of our securities or assets, obtaining loans from financial institutions or entering into partnership arrangements. Our continued net operating losses increase the difficulty in obtaining such capital, and there can be no assurances that we will be able to obtain such capital on favorable 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 clinical development

programs or commercialization efforts, and/or ultimately cease operations.

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, 2017, we had an accumulated deficit of \$303.1 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, sales of our



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common stock pursuant to our at-the-market equity offering programs, and our loan and security agreement with Hercules Capital Inc. (Hercules). The proceeds of our term loan facility with Hercules, which we entered into in March 2017 and amended in January and March 2018, will be used for our ongoing research and development programs and for general corporate purposes. As of December 31, 2017, there was \$35.0 million available to borrow under the amended term loan facility with Hercules, subject to certain conditions of funding. We have devoted substantially all of our efforts to research and development. 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:

- prepare for the anticipated commercialization of duvelisib;
- continue our ongoing clinical trials with our product candidates, including with our most advanced product candidates duvelisib and defactinib;
- initiate additional clinical trials for our product candidates;
- maintain, expand and protect our intellectual property portfolio;
- acquire or in-license other products and technologies;
- hire additional clinical, development and scientific personnel;
  - add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts; and
- establish a sales, marketing and distribution infrastructure to commercialize any products for which we obtain marketing approval.

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, including for duvelisib.

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the clinical development of our product candidates and as we seek marketing approval for duvelisib. If we receive such approval, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution of duvelisib. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations, including for our clinical development programs and any commercialization efforts for duvelisib.

We expect our existing cash, cash equivalents and investments will enable us to fund our current operating plan and capital expenditure requirements into the second half of 2018. Our future capital requirements will depend on many factors, including:

- the scope, progress and results of our ongoing and potential future clinical trials;
- the extent to which we acquire or in license other product candidates and technologies;
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- the costs, timing and outcome of regulatory review of our product candidates (including our efforts to seek approval and fund the preparation and filing of regulatory submissions);
- the costs and timing of commercialization activities for the product candidates for which we expect to receive marketing approval;
  - revenue, if any, received from commercial sales of our product candidates, should any of our product candidates receive marketing approval;

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- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property related claims; and
- our ability to establish collaborations 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 of any of our product candidates, including duvelisib. Though we submitted an NDA for duvelisib in February 2018, the NDA may not be accepted or approved by the FDA, and even if approved, duvelisib may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of products, such as duvelisib, that may not be commercially available for several years, if at all. Accordingly, even if we receive regulatory approval of one of our product candidates, such as duvelisib, 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 or entering into certain licensing arrangements 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. 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. To the extent that we enter into certain licensing arrangements, the ownership interest of our existing stockholders may be diluted if we elect to make certain payments in shares of our common stock. For example, pursuant to the terms of our license agreement with Infinity, we may elect to make certain milestone payments in shares of common stock in lieu of cash, according to a formula set forth in the license agreement. 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. For example, see our risk factors under the heading “Risks Related to Our Indebtedness.”

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 INDEBTEDNESS

Our level of indebtedness and debt service obligations could adversely affect our financial condition, and may make it more difficult for us to fund our operations.

In March 2017, we entered into a Loan and Security Agreement with Hercules, which was subsequently amended in January and March 2018. Under the Loan and Security Agreement, as amended (the Amended Loan Agreement), Hercules will provide access to term loans with an aggregate principal amount of up to \$50.0 million. Under the Amended Loan Agreement, we borrowed an initial tranche of \$2.5 million in March 2017, we drew an additional \$7.5

million in October 2017, and in December 2017 we drew an additional \$5.0 million.

All obligations under the Amended Loan Agreement are secured by substantially all of our existing property and assets, excluding our intellectual property. This indebtedness may create additional financing risk for us, particularly if our business or prevailing financial market conditions are not conducive to paying off or refinancing our outstanding debt obligations at maturity. This indebtedness could also have important negative consequences, including:

- we will need to repay our indebtedness by making payments of interest and principal, which will reduce the amount of money available to finance our operations, our research and development efforts and other general corporate activities; and
- our failure to comply with the restrictive covenants in the Amended Loan Agreement could result in an

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event of default that, if not cured or waived, would accelerate our obligation to repay this indebtedness, and Hercules could seek to enforce their security interest in the assets securing such indebtedness.

To the extent additional debt is added to our current debt levels, the risks described above could increase.

We may not have cash available in an amount sufficient to enable us to make interest or principal payments on our indebtedness when due.

Failure to satisfy our current and future debt obligations under the Amended Loan Agreement, or breaching any covenants under the Amended Loan Agreement, subject to specified cure periods with respect to certain breaches, could result in an event of default and, as a result, Hercules could accelerate all of the amounts due. In the event of an acceleration of amounts due under the Amended Loan Agreement as a result of an event of default, we may not have enough available cash or be able to raise additional funds through equity or debt financings to repay such indebtedness at the time of such acceleration. In that case, we may be required to delay, limit, reduce or terminate our product candidate development or commercialization efforts or grant to others rights to develop and market product candidates that we would otherwise prefer to develop and market internally. Hercules could also exercise its rights as collateral agent to take possession and dispose of the collateral securing the term loans for its benefit, which collateral includes substantially all of our property other than our intellectual property. Our business, financial condition and results of operations could be materially adversely affected as a result of any of these events. We are subject to certain restrictive covenants which, if breached, could have a material adverse effect on our business and prospects.

The Amended Loan Agreement imposes operating and other restrictions on us. Such restrictions will affect, and in many respects limit or prohibit, our ability and the ability of any future subsidiary to, among other things:

- dispose of certain assets;
- change our lines of business;
- engage in mergers, acquisitions or consolidations;
- incur additional indebtedness;
- create liens on assets;
- pay dividends and make distributions or repurchase our capital stock; and
- engage in certain transactions with affiliates.

### RISKS RELATED TO OUR DEPENDENCE ON THIRD PARTIES

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 (CROs), clinical data management organizations, medical institutions and clinical investigators, to conduct, provide monitors for and manage data from all of our clinical trials. 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 and other regulatory agencies require us to comply with standards, commonly referred to as Good Clinical Practices (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

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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 government sponsored databases, such as 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 our 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 our product candidates.

We intend to rely on academic and private non-academic institutions to conduct and sponsor clinical trials relating to our 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 firsthand 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



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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 (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.

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.

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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 our product candidates 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.

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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 Infinity and Pfizer, and expect to enter into additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, milestone payment, royalty, insurance and other obligations on us. For example, under our license agreements with Infinity and Pfizer, we are required to use diligent or 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, which may not be possible. If Pfizer were to terminate its license agreement with us for any reason, we would lose our rights to defactinib. If Infinity were to terminate its license agreement with us for any reason, we would lose our rights to duvelisib.

If we are unable to obtain and maintain patent protection for our products, or if our licensors are unable to obtain and maintain patent protection for the 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 products similar or identical to ours, and our ability to successfully commercialize our 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 products. We and our licensors seek to protect our proprietary position by filing patent applications in the United States and abroad related to our products that are important to our business. We cannot be certain that any patents will issue with claims that cover our 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 products that we license from third parties and are reliant on our licensors. 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 products or which effectively prevent others from commercializing competitive 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 pre

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issuance 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 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 products, or limit the duration of the patent protection of our 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 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, 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. 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 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

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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 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.



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**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 for duvelisib, or any of our product candidates, we will not be able to commercialize duvelisib, or any such other candidates, and our ability to generate revenue will be materially impaired.

Though we submitted an NDA for duvelisib in February 2018, the NDA may not be accepted or approved by the FDA. Obtaining approval of an NDA can be a lengthy, expensive and uncertain process. Duvelisib and the activities associated with its development and commercialization, including its design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, as with our other product candidates, 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 duvelisib will prevent us from commercializing duvelisib. 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. Duvelisib 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 subject to more limited indications than those we propose 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 duvelisib, its commercial prospects 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 United States and the European Union for the use of duvelisib in CLL/SLL and FL, in the United States and European Union for the use of defactinib in ovarian cancer, and the United States, the European Union, and Australia for the use of defactinib in mesothelioma. If duvelisib or defactinib 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 (FDCA), up to ten years of marketing exclusivity in Europe, and five years of marketing exclusivity in Australia. A competitor may receive orphan drug marketing authorization prior to us for the same indication for which we are developing duvelisib or defactinib. Other

companies have received orphan drug designations for compounds other than duvelisib or defactinib for the same indications for which we may have received orphan drug designation in corresponding territories. While orphan drug exclusivity for duvelisib or defactinib would provide market exclusivity against the same active ingredient for the same indication, 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 exclusivity. Furthermore, the marketing exclusivity in Europe can be reduced from ten 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 active ingredient and same indication before we do. If that were to

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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 the FDA may approve a competing product for the same indication during the seven-year period of marketing exclusivity, such as if the later product is the same compound as our product but 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 of another compound for the same use as the orphan drug.

Though we have received fast track designation by the FDA for duvelisib in certain indications, that designation may not actually lead to a faster development or regulatory review or approval process, and it does not ensure that we will receive marketing approval.

The FDA has granted fast track designation to the investigation of duvelisib for the treatment of patients with FL who have received at least two prior therapies and for the potential treatment of patients with CLL who have received at least one prior therapy. Any drug sponsor may apply for such designation if its product candidate is intended for the treatment of a serious or life-threatening disease or condition and the product candidate demonstrates the potential to address an unmet medical need. The FDA has broad discretion whether or not to grant fast track designation. Although duvelisib has received such designation, this may not actually result in a faster development process, review or approval compared to standard FDA procedures. The FDA may withdraw fast track designation if it believes that the clinical development program does not continue to meet the criteria for fast track designation.

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, for example, if we obtain marketing approval for duvelisib, 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, including the imposition of a REMS. 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;

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- refusal to permit the import or export of our products;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

The FDA's and other regulatory authorities' policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may fail to obtain any marketing approvals, lose any marketing approval that we may have obtained and we may not achieve or sustain profitability.

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. A person or entity does not need to have actual knowledge of the anti-kickback statute or specific intent to violate it in order to have committed a violation;
- the federal False Claims Act (FCA) imposes criminal and civil penalties on 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 and actions under the FCA may be brought by private whistleblowers as well as the government. In addition, the government may assert that a claim including items and services resulting from a violation of the federal anti-kickback statute constitutes a false or fraudulent claim for purposes of the FCA;
- the federal Health Insurance Portability and Accountability Act of 1996 (HIPAA) imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program and HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, also establishes requirements related to the privacy, security and transmission of individually identifiable health information which apply to many healthcare providers, physicians and third-party payors with whom we interact;
- 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 FDCA, which among other things, strictly regulates drug product and medical device marketing, prohibits manufacturers from marketing such products for off-label use and regulates the distribution of samples;
- federal laws that require pharmaceutical manufacturers to report certain calculated product prices to the government or provide certain discounts or rebates to government authorities or private entities, often as a condition of reimbursement under governmental healthcare programs;
- the so-called federal "sunshine law" or Open Payments requires manufacturers of drugs, devices, biologics and medical supplies to report to the Department of Health and Human Services information related to payments and other transfers of value to physicians and teaching hospitals, as well as 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-

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governmental third-party payors, including private insurers, and some state laws regulate interactions between pharmaceutical companies and healthcare providers and 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 healthcare providers or marketing expenditures and pricing information. State laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

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, including arrangements we may have with physicians and other healthcare providers, 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, and we expect there will continue to be, a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could, among other things, 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.

The U.S. healthcare industry generally and U.S. government healthcare programs in particular are highly regulated and subject to frequent and substantial changes. The U.S. government and individual states have been aggressively pursuing healthcare reform. For example, in March 2010, President Obama signed into law the Health Care Reform Act, 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 healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The law, for example, increased drug rebates under state Medicaid programs for brand name prescription drugs and extending those rebates to Medicaid managed care and assessed a fee on manufacturers and importers of brand name prescription drugs reimbursed under certain government programs, including Medicare and Medicaid.



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Since its enactment, there have been ongoing judicial, legislative and administrative efforts to modify, repeal or prevent implementation of various provisions of the Health Care Reform Act. See “GOVERNMENT REGULATION – New Legislation and Regulations.” We cannot predict the ultimate content, timing or effect of any federal or state healthcare reform legislation or the impact of potential legislation on us.

In addition, other legislative changes have been proposed and adopted in the U.S. since the Health Care Reform Act was enacted. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and, due to the Bipartisan Budget Act of 2015, will remain in effect through 2025 unless additional action is taken by Congress. Recent tax reform legislation eliminates the tax penalty for individuals who do not maintain sufficient health insurance coverage beginning in 2019 (the so-called “individual mandate”).

Individual states in the United States have also become increasingly active in passing legislation and implementing regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, measures designed to encourage importation from other countries and bulk purchasing. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs.

We cannot be sure whether additional legislative changes will be enacted, or whether the 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, Daniel Paterson, our Chief Operating Officer, and Joseph Lobacki, our Chief Commercial Officer, as well as the other principal members of our management and scientific teams. Although we have formal employment agreements with Robert Forrester, Daniel Paterson, and Joseph Lobacki, 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, universities and research institutions for similar personnel. Although we have implemented a retention plan for certain key employees, our retention plan may not be successful in incentivizing these employees to continue their employment with us. 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.



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

We may experience significant growth over time 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 may 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 when we expand. 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 breaches or 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 key business processes and 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 be exposed to liability, which could have a material adverse effect on our operating results and financial condition and possibly delay the further development and commercialization of our product candidates.

## 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.

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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 \$1.05 through February 28, 2018. 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.

Failure to comply with The Nasdaq Global Market continued listing requirements may result in our common stock being delisted from The Nasdaq Global Market.

If our stock price falls below \$1.00 per share, we may not continue to qualify for continued listing on The Nasdaq Global Market. To maintain listing, we are required, among other things, to maintain a minimum closing bid price of \$1.00 per share. If the closing bid price of our common stock is below \$1.00 per share for 30 consecutive business days, we will receive a deficiency notice from Nasdaq advising us that we have a certain period of time, typically 180 days, to regain compliance by maintaining a minimum closing bid price of at least \$1.00 for at least ten consecutive business days, although Nasdaq could require a longer period.

The delisting of our common stock would significantly affect the ability of investors to trade our common stock and negatively impact the liquidity and price of our common stock. In addition, the delisting of our common stock could materially adversely impact our ability to raise capital on acceptable terms or at all. Delisting from The Nasdaq Global Market could also have other negative results, including the potential loss of confidence by our current or prospective

third-party providers and collaboration partners, the loss of institutional investor interest, and fewer licensing and partnering opportunities.

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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 current or 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.

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, 2017	High	Low
First quarter	\$ 2.25	\$ 1.11
Second quarter	\$ 2.54	\$ 1.61
Third quarter	\$ 5.71	\$ 2.11
Fourth quarter	\$ 4.92	\$ 2.95

Year ended December 31, 2016	High	Low
First quarter	\$ 1.89	\$ 1.05
Second quarter	\$ 1.93	\$ 1.19

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Third quarter	\$ 1.66	\$ 1.27
Fourth quarter	\$ 1.55	\$ 1.05

HOLDERS

As of February 28, 2018, there were 16 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 \$3.06. 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.



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## 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 of 1933, as amended, 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 December 31, 2012 through December 31, 2017. The comparison assumes \$100 was invested after the market closed on December 31, 2012 in our common stock and in each of the foregoing indices, and it assumes reinvestment of dividends, if any.

## COMPARISON OF 5 YEAR CUMULATIVE TOTAL RETURN\*

Among Verastem, Inc., the Nasdaq Composite Index, and the Nasdaq Biotechnology Index

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\*\$100 invested on 12/31/12 in stock or index, including reinvestment of dividends. Fiscal year ending December 31, 2017.

## Cumulative Total Return Comparison

	December 31,					
	2012	2013	2014	2015	2016	2017
Verastem, Inc.	100.00	129.69	103.98	21.16	12.74	34.93
Nasdaq Composite	100.00	141.63	162.09	173.33	187.19	242.29
Nasdaq Biotechnology	100.00	174.05	230.33	244.29	194.95	228.29

## 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.

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## 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:	Year ended December 31,				
	2017	2016	2015	2014	2013
	(in thousands, except share and per share amounts)				
Operating expenses:					
Research and development	\$ 46,423	\$ 19,779	\$ 40,565	\$ 35,448	\$ 25,930
General and administrative	21,381	17,223	17,634	18,159	15,472
Total operating expenses	67,804	37,002	58,199	53,607	41,402
Loss from operations	(67,804)	(37,002)	(58,199)	(53,607)	(41,402)
Interest income	561	562	334	242	200
Interest expense	(559)	—	—	—	—
Net loss applicable to common stockholders	\$ (67,802)	\$ (36,440)	\$ (57,865)	\$ (53,365)	\$ (41,202)
Net loss per share applicable to common stockholders—basic and diluted	\$ (1.76)	\$ (0.99)	\$ (1.61)	\$ (2.07)	\$ (1.82)
Weighted average number of common shares used in net loss per share applicable to common stockholders—basic and diluted	38,422	36,988	35,932	25,804	22,680
Balance sheet data:	As of December 31,				
	2017	2016	2015	2014	2013
	(in thousands)				
Cash, cash equivalents and investments	\$ 86,672	\$ 80,897	\$ 110,258	\$ 92,675	\$ 123,656
Working capital	70,659	70,304	100,734	86,112	94,151
Total assets	89,791	83,629	113,094	98,649	125,261
Accumulated deficit	(303,142)	(235,323)	(198,883)	(141,018)	(87,653)
Total stockholders’ equity	57,684	72,297	102,469	88,766	117,446

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### Item 7. Management’s Discussion and Analysis of Financial Condition and Results of Operations

You should read the following discussion and analysis of our financial condition and results of operations together with our 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 the heading “Forward Looking Statements.”

#### OVERVIEW

We are a biopharmaceutical company focused on developing and commercializing drugs to improve with the survival and quality of life of cancer patients. Our most advanced product candidates, duvelisib and defactinib, utilize a multi-faceted approach to treat cancers originating either in the blood or major organ systems. We are currently evaluating these compounds in both preclinical and clinical studies as potential therapies for certain cancers, including leukemia, lymphoma, lung cancer, ovarian cancer, mesothelioma, and pancreatic cancer. We believe that these compounds may be beneficial as therapeutics either as single agents or when used in combination with immuno-oncology agents or other current and emerging standard of care treatments in aggressive cancers that are poorly served by currently available therapies.

Duvelisib targets the Phosphoinositide 3-kinase (PI3K) signaling pathway. The PI3K signaling pathway plays a central role in cancer proliferation and survival. Duvelisib is an investigational oral therapy designed to attack both malignant B- and T-cells and disrupt the tumor microenvironment to help thwart their growth and proliferation through the dual inhibition of PI3K delta and gamma. Duvelisib is being developed for the treatment of patients with hematologic cancers including chronic lymphocytic leukemia and small lymphocytic lymphoma (CLL/SLL) and indolent non-Hodgkin lymphoma (iNHL), which includes follicular lymphoma (FL), and other subtypes of lymphoma, including peripheral T-cell lymphoma (PTCL). Duvelisib has U.S. Food and Drug Administration (FDA) Fast Track Designation for patients with CLL or PTCL who have received at least one prior therapy and for patients with FL who have received at least two prior therapies. In addition, duvelisib has orphan drug designation for patients with CLL/SLL and FL in the United States and European Union.

Duvelisib was evaluated in late- and mid-stage clinical trials, including DUO™, a randomized, Phase 3 monotherapy study in patients with relapsed or refractory CLL/SLL, and DYNAMO™, a single-arm, Phase 2 monotherapy study in patients with double-refractory iNHL, including FL, SLL, and marginal zone lymphoma (MZL). Both DUO and DYNAMO achieved their primary endpoints upon top-line analysis of efficacy data. We submitted a New Drug Application (NDA) to the FDA requesting the full approval of duvelisib for the treatment of patients with relapsed or refractory CLL/SLL and accelerated approval for the treatment of patients with relapsed or refractory FL in February 2018.

Defactinib is a targeted inhibitor of the Focal Adhesion Kinase (FAK) signaling pathway. FAK is a non-receptor tyrosine kinase encoded by the PTK-2 gene that is involved in cellular adhesion and, in cancer, metastatic capability. Similar to duvelisib, defactinib is also orally available and designed to be a potential therapy for patients to take at home under the advice of their physician. Defactinib has orphan drug designation in ovarian cancer in the United States and the European Union, and in mesothelioma in the United States, the European Union, and Australia.



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Defactinib is currently being evaluated in a Phase 1b study in combination with Merck & Co.'s PD-1 inhibitor pembrolizumab and gemcitabine in patients with advanced pancreatic cancer, a Phase 1/2 clinical collaboration with Pfizer Inc. (Pfizer) and Merck KGaA to evaluate defactinib in combination with avelumab, an anti-PD-L1 antibody, in patients with ovarian cancer, and a Phase 1/2 study in collaboration with Cancer Research UK and Merck & Co. for the combination of defactinib with pembrolizumab in patients with non-small cell lung cancer (NSCLC), mesothelioma or pancreatic cancer.

Our operations to date have been organizing and staffing our company, business planning, raising capital, identifying and acquiring potential product candidates and undertaking preclinical studies and clinical trials for our product candidates. To date, we have not generated any revenues. We have financed our operations to date through private placements of preferred stock, public offerings of our common stock, sales of common stock under our at-the-market equity offering programs, and our loan and security agreement executed with Hercules Capital, Inc. (Hercules) in March 2017, as amended.

As of December 31, 2017, we had an accumulated deficit of \$303.1 million. Our net loss was \$67.8 million, \$36.4 million and \$57.9 million for the years ended December 31, 2017, 2016 and 2015, 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 seek marketing approval for our lead product candidate, duvelisib, and continue the research and development and clinical trials of all of 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 will depend heavily on the successful development and potential commercialization of our product candidates.

### Research and development expenses

Research and development expenses consist of costs associated with our research activities, including 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 (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.

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We 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, including duvelisib and defactinib, for the years ended December 31, 2017, 2016 and 2015. We use our employee and infrastructure resources across multiple research and development projects. 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 \$5.8 million, \$3.9 million and \$7.3 million of personnel costs for the years ended December 31, 2017, 2016 and 2015, respectively.

	Year ended December 31,		
	2017 (in thousands)	2016 (in thousands)	2015 (in thousands)
Duvelisib	\$ 30,409	\$ 3,326	\$ —
Defactinib	2,894	3,934	20,713
Unallocated and other research and development expense	11,739	11,445	17,442
Unallocated stock-based compensation expense	1,381	1,074	2,410
Total research and development expense	\$ 46,423	\$ 19,779	\$ 40,565

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

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

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administrative expenses include allocated facility costs and professional fees for legal, patent, investor and public relations, consulting, insurance premiums, audit, tax and other public company costs.

### Interest income and interest expense

Interest income reflects interest earned on our cash, cash equivalents and available-for-sale securities.

Interest expense reflects interest expense due under our term loan facility executed with Hercules and non-cash interest related to the amortization of debt discount and issuance costs.

## 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 (GAAP). The preparation of these consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of certain 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 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 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.

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### Stock based compensation

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 Securities and Exchange Commission Staff Accounting Bulletin 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. Our current computation of expected volatility is based on the historical volatility of five companies, including our own and a representative group of four public biotechnology and life sciences companies with similar characteristics to us, including similar stage of product development and therapeutic focus. 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. Historically, we have recognized stock-based compensation net of estimated forfeitures over the vesting period of the respective grant. Effective January 1, 2017, we adopted Accounting Standard Updated (ASU) 2016-09, Compensation – Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting, which simplified the accounting for stock-based compensation arrangements, including the accounting for forfeitures. Upon adoption, we elected to begin accounting for forfeitures as they occur, rather than estimating a forfeiture rate, and recorded an immaterial cumulative-effect adjustment to opening accumulated deficit.

We have also granted performance based restricted stock units (RSUs) and stock options 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 and stock options 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 and stock options 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, 2017, there was approximately \$6.8 million of unrecognized stock based compensation related to stock options, which are expected to be recognized over a weighted average period of 2.9 years. There is no unrecognized stock based compensation related to RSUs or restricted common stock. 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.

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RESULTS OF OPERATIONS

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

Comparison of the Year Ended December 31, 2017 to the Year Ended December 31, 2016

**Research and development expense.** Research and development expense for the year ended December 31, 2017 (2017 Period) was \$46.4 million compared to \$19.8 million for the year ended December 31, 2016 (2016 Period). The \$26.6 million increase from the 2016 Period to the 2017 Period was primarily related to an increase of \$13.4 million in external CRO expense for outsourced biology, chemistry, development and clinical services, which includes our clinical trial costs, the achievement of a \$6.0 million milestone pursuant to our license agreement with Infinity, an increase of \$5.1 million in consulting fees, an increase in personnel related costs of \$1.9 million, and a net increase of approximately \$243,000 in stock-based compensation and other expenses.

**General and administrative expense.** General and administrative expense for the 2017 Period was \$21.4 million compared to \$17.2 million for the 2016 Period. The increase of \$4.2 million from the 2016 Period to the 2017 Period primarily resulted from increases in consulting and professional fees of \$4.4 million, including \$2.5 million related to commercial launch preparation, an increase in personnel costs of \$1.0 million and an increase in facilities and other expenses of approximately \$200,000. These increases were partially offset by a decrease in stock-based compensation expense of \$1.5 million.

**Interest income.** Interest income remained flat from the 2016 Period to the 2017 Period primarily as a result of higher interest rates on investments in the 2017 Period, offset by a lower investment cost basis.

**Interest expense.** Interest expense for the 2017 Period was approximately \$559,000 and related to our loan and security agreement executed with Hercules in March 2017, as amended. We did not incur any interest expense in the 2016 Period.

Comparison of the Year Ended December 31, 2016 to the Year Ended December 31, 2015

**Research and development expense.** Research and development expense for the 2016 Period was \$19.8 million compared to \$40.6 million for the year ended December 31, 2015 (2015 Period). The \$20.8 million decrease from the 2015 Period to the 2016 Period was primarily related to a decrease of \$15.6 million in external CRO expense for outsourced biology, chemistry, development and clinical services, which includes our clinical trial costs, a \$3.4 million decrease in personnel related costs, primarily due to the reduction in workforce in October 2015, a decrease of \$1.3 million in stock-based compensation expense and a decrease of \$1.5 million in lab supplies, travel and other research and development expense. These decreases were partially offset by an increase of approximately \$947,000 in consulting and professional fees.

**General and administrative expense.** General and administrative expense for the 2016 Period was \$17.2 million compared to \$17.6 million for the 2015 Period. The approximate \$411,000 decrease from the 2015 Period to the 2016 Period primarily resulted from a decrease of \$2.1 million in stock-based compensation expense. This decrease was partially offset by increases of \$1.1 million in consulting and professional fees, approximately \$280,000 in personnel costs, and a net increase of approximately \$306,000 of other general and administrative costs.

**Interest income.** Interest income increased to approximately \$562,000 for the 2016 Period from approximately \$334,000 for the 2015 Period. This increase was primarily due to higher interest rates on investments in the 2016 Period compared to the 2015 Period.



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## 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 preferred stock, public offerings of our common stock, sales of common stock under our at-the market equity offering programs, and our loan and security agreement executed with Hercules in March 2017, as amended.

As of December 31, 2017, we had \$86.7 million in cash, cash equivalents and investments. We primarily invest our cash, cash equivalents and investments in a U.S. Government money market fund and corporate bonds and commercial paper of publicly traded companies.

## Cash flows

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

	Year ended December 31,		
	2017	2016	2015
Net cash (used in) provided by:			
Operating activities	\$ (57,310)	\$ (29,484)	\$ (45,559)
Investing activities	43,953	36,968	(27,057)
Financing activities	63,184	(5)	63,585
Increase in cash and cash equivalents	\$ 49,827	\$ 7,479	\$ (9,031)

**Operating activities.** The use of cash in operating activities for all periods resulted primarily from our net losses adjusted for non-cash charges and changes in the components of working capital. The \$27.8 million increase in cash used in operating activities for the 2017 Period compared to the 2016 Period was primarily due to an increase in research and development expenses related to our license agreement with Infinity, including our ongoing clinical trials and the achievement of a \$6.0 million milestone in the 2017 Period. The \$16.1 million decrease in cash used in operating activities for the 2016 Period compared to the 2015 Period was primarily due to a decrease in research and development expenses related to our ongoing clinical trials, including the closeout of our COMMAND trial, and development of our lead product candidates.

**Investing activities.** The cash provided by investing activities for the 2017 Period reflects net maturities of investments of \$44.0 million. The cash provided by investing activities for the 2016 Period reflects net maturities of investments of \$37.0 million. The cash used for investing activities for the 2015 Period primarily reflects the net purchases of investments of \$26.8 million.

**Financing activities.** The cash provided by financing activities for the 2017 Period primarily represents \$24.7 million in net proceeds received from an underwritten offering of 8,422,877 shares of our common stock at a price of \$2.97 per share with BTIG, LLC, \$23.1 million in net proceeds received under our at-the-market equity program, \$14.8 million in net proceeds received from a loan and security agreement executed with Hercules, and approximately \$442,000 received from the exercise of stock options, offset by approximately \$138,000 of deferred financing costs. The cash used in financing activities for the 2016 Period primarily represents approximately \$5,000 used to satisfy the tax withholding obligations on certain RSUs that were net settled by employees. The cash provided by financing activities for the 2015 Period primarily represents net proceeds of \$63.9 million from the sale of shares of our common stock in our January 2015 follow-on offering and our at-the-market equity offering program, offset in part by approximately \$417,000 of cash used to satisfy the tax withholding obligations on certain RSUs that were net settled

by employees.

On March 21, 2017 (Closing Date), we entered into a term loan facility of up to \$25.0 million with Hercules, a Maryland corporation, the proceeds of which will be used for its ongoing research and development programs and for general corporate purposes. The term loan facility is governed by a loan and security agreement, dated March 21, 2017 (the Original Loan Agreement), which originally provided for up to four separate advances, of which the first tranche of \$2.5 million was drawn on the Closing Date. The second and third tranches of \$2.5

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million and \$5.0 million, were both drawn on October 12, 2017 after announcing favorable data from our Phase III clinical study evaluating the safety and efficacy of duvelisib in patients with relapsed/refractory CLL/SLL. A total of \$6.0 million of the proceeds received from the second and third tranches were used to make a milestone payment pursuant to our license agreement with Infinity. The fourth tranche consisted of \$15.0 million that could be drawn, at our option and at the sole discretion of Hercules, on or prior to June 30, 2018. On December 20, 2017, we drew an advance under the fourth tranche of \$5.0 million.

On January 4, 2018, we entered into the First Amendment to the Original Loan Agreement (the First Amendment) and on March 6, 2018, we entered into the Second Amendment to the Original Loan Agreement (the Second Amendment, and the Original Loan Agreement as amended by the First Amendment and the Second Amendment, the Amended Loan Agreement). The First Amendment increased the total borrowing limit under the Original Loan Agreement from up to \$25.0 million to up to \$50.0 million (the Term Loan). As \$15.0 million in term loans had already been drawn prior to entering into the First Amendment, there is \$35.0 million of borrowing capacity remaining under the Amended Loan Agreement. The remaining \$35.0 million of borrowing capacity may be drawn in minimum increments of \$5.0 million in multiple tranches comprised of (i) term loans (each a Term E Loan Advance) in an aggregate principal amount of up to \$10.0 million and (ii) subject to Hercules' sole discretion, term loans (each a Term F Loan Advance) in an aggregate principal amount of up to \$25.0 million. The Amended Loan Agreement permits us to draw Term E Loan Advances subject to (i) the FDA accepting on or prior to September 30, 2018 our NDA for duvelisib and (ii) delivery of our financial and business projections to Hercules in form and substance reasonably acceptable to Hercules. In addition, the Amended Loan Agreement allows us to draw Term F Loan Advances subject to the prior drawing of all other tranches and Hercules' sole discretion.

The Term Loan will mature on December 1, 2020 (Loan Maturity Date). Each advance accrues interest at a floating per annum rate equal to the greater of either (a) 10.5% or (b) the lesser of (i) 12.75% and (ii) the sum of (x) 10.5% plus (y) (A) the prime rate minus (B) 4.5%. The Term Loan provided for interest-only payments until November 1, 2018, which was extended to May 1, 2019 pursuant to the Amended Loan Agreement upon our receipt of a minimum of \$20.0 million cash proceeds from a sale of equity securities in December 2017. Thereafter, amortization payments will be payable monthly in twenty installments of principal and interest (subject to recalculation upon a change in prime rates). Any advance may be prepaid in whole or in part upon seven business days' prior written notice to Hercules, subject to a prepayment charge of 3.0%, if such advance is prepaid in any of the first twelve (12) months following the Closing Date, 2.0%, if such advance is prepaid after twelve (12) months following the Closing Date but on or prior to twenty-four (24) months following the Closing Date, and 1.0% thereafter. In addition, a final payment equal to 4.5% of the greater of (a) \$5.0 million and (b) the total principal amount of the Term Loan extended by Hercules which is due on the Loan Maturity Date, or such earlier date specified in the Amended Loan Agreement. Amounts outstanding during an event of default shall be payable on demand and shall accrue interest at an additional rate of 5.0% per annum of the past due amount outstanding.

The Term Loan is secured by a lien on substantially all of the assets of Verastem, Inc., other than intellectual property and contains customary covenants and representations, including a liquidity covenant, financial reporting covenant and limitations on dividends, indebtedness, collateral, investments, distributions, transfers, mergers or acquisitions, taxes, corporate changes, deposit accounts, and subsidiaries.

The events of default under the Amended Loan Agreement include, without limitation, and subject to customary grace periods, (1) any failure by us to make any payments of principal or interest under the Amended Loan Agreement, promissory notes or other loan documents, (2) any breach or default in the performance of any covenant under the Amended Loan Agreement, (3) any making of false or misleading representations or warranties in any material respect, (4) our insolvency or bankruptcy, (5) certain attachments or judgments on the assets of Verastem, Inc., or (6) the occurrence of any material default under certain agreements or obligations of ours involving indebtedness, or (7) the occurrence of a material adverse effect. If an event of default occurs, Hercules is entitled to take enforcement action, including acceleration of amounts due under the Amended Loan Agreement.

The Amended Loan Agreement also contains other customary provisions, such as expense reimbursement and confidentiality. Hercules has indemnification rights and the right to assign the Term Loan.

In December 2013, we established an at-the-market equity offering program pursuant to which we were able to offer and sell up to \$35.0 million of our common stock at then-current market prices from time to time

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through Cantor Fitzgerald & Co. (Cantor), as sales agent (the 2013 ATM Program). In November 2014, we commenced sales under the 2013 ATM Program. During the year ended December 31, 2015, we sold 1,189,479 shares of common stock under the 2013 ATM Program with net proceeds (after deducting commissions and other offering expenses) of \$12.9 million. No proceeds were received and no additional sales of our common stock were made under the 2013 ATM Program during the years ended December 31, 2017 and 2016.

On March 30, 2017, we terminated the 2013 ATM Program and established a new at-the-market equity offering program pursuant to which we were able to offer and sell up to \$35.0 million of our common stock at then-current market prices from time to time through Cantor, as sales agent (the 2017 ATM Program). On August 28, 2017, we amended our sales agreement with Cantor to increase the maximum aggregate offering price of shares of common stock that can be sold under the 2017 ATM Program to \$75.0 million. Through December 31, 2017, we sold 5,036,879 shares under the 2017 ATM Program for net proceeds of approximately \$23.1 million (after deducting commissions and other offering expenses).

As of March 13, 2018, we sold an additional 97,078 shares of common stock under the at-the-market equity offering program with net proceeds of approximately \$342,000 (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 \$50.9 million, after deducting underwriting discounts and commissions.

Funding requirements

We expect to continue to incur significant expenses and operating losses for the foreseeable future. We anticipate that our expenses and operating losses will increase substantially if and as we:

- prepare for the anticipated commercialization of duvelisib;
- continue our ongoing clinical trials, including with our most advanced product candidates duvelisib and defactinib;

add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts; and

establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval.

Without additional funding, we do not believe that we have sufficient funds to meet our obligations within the next twelve months from the date of issuance of these consolidated financial statements. These factors raise substantial doubt about our ability to continue as a going concern. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates, and the extent to which we may enter into collaborations with third parties for development and commercialization of our product candidates, we are unable to estimate the amounts of increased capital outlays and operating expenses associated with completing the development of our current product candidates. Our future capital requirements will depend on many factors, including:

- the scope, progress and results of our 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 (including our efforts to seek approval and fund the preparation and filing of regulatory submissions);

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the costs and timing of commercialization activities for our product candidates, for which we receive marketing approval;  
 revenue, if any, received from commercial sales of our product candidates, should any of our product candidates receive marketing approval;  
 the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims; and  
 our ability to establish collaborations on favorable terms, if at all.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. 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, 2017:

(in thousands)	Total	2018	2019 - 2020	2021 -	2022	Thereafter
Operating lease obligations	\$ 957	\$ 542	\$ 415	\$ —	\$ —	
Amended Loan Agreement	15,000	—	15,000	—	—	
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 may require us to pay upfront license fees, ongoing annual license maintenance fees, milestone payments, minimum royalty payments, as well as reimbursement of certain patent costs incurred by the licensors, as applicable. We have not included these payments in the table above because: there were no upfront license fees payable in future periods; no annual license maintenance fees; we cannot estimate if milestone and/or royalty payments will occur in future periods; and patent cost reimbursement costs are perpetual and the agreements are cancelable by us at any time upon prior written notice to the licensor.

In November 2016, we entered into an amended and restated license agreement with Infinity, under which we acquired an exclusive worldwide license for the research, development, commercialization, and manufacture of products in oncology indications containing duvelisib. In connection with the license agreement, we assumed operational and financial responsibility for certain activities that were part of Infinity's duvelisib program, including the DUO study for patients with relapsed/refractory CLL/SLL, and Infinity assumed financial responsibility for the shutdown of certain other clinical studies up to a maximum of \$4.5 million. We are obligated to use diligent efforts to develop and commercialize a product in an oncology indication containing duvelisib. During the term of the license agreement, Infinity has agreed not to research, develop, manufacture or commercialize duvelisib in any other

indication in humans or animals.

Pursuant to the terms of the license agreement, we are required to make the following payments to Infinity in cash or, at our election, in whole or in part, in shares of our common stock: (i) \$6.0 million upon the completion of the DUO study if the results of the DUO study meet certain pre-specified criteria, which milestone was paid in

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cash by us to Infinity in October 2017, and (ii) \$22.0 million upon the approval of an NDA in the United States or an application for marketing authorization with a regulatory authority outside of the United States for a product in an oncology indication containing duvelisib. For any portion of any of the foregoing payments that we elect to issue in shares of our common stock in lieu of cash, the number of shares of common stock to be issued will be determined by multiplying (1) 1.025 by (2) the number of shares of common stock equal to (a) the amount of the payment to be paid in shares of common stock divided by (b) the average closing price of a share of common stock as quoted on Nasdaq for a twenty day period following the public announcement of the applicable milestone event. The shares of common stock will be issued as unregistered securities, and we will have an obligation to promptly file a registration statement with the SEC to register such shares for resale. Any issuance of shares will be subject to the satisfaction of closing conditions, including that all material authorizations, consents, approvals and the like necessary for such issuance shall have been obtained.

We are also obligated to pay Infinity royalties on worldwide net sales of any products in an oncology indication containing duvelisib ranging from the mid-single digits to the high single-digits. The royalties will expire on a product-by-product and country-by-country basis until the latest to occur of (i) the last-to-expire patent right covering the applicable product in the applicable country, (ii) the last-to-expire patent right covering the manufacture of the applicable product in the country of manufacture of such product, (iii) the expiration of non-patent regulatory exclusivity in such country and (iv) ten years following the first commercial sale of a product in a country, provided that if royalties on net sales for a product in the United States are payable solely on the basis of non-patent regulatory exclusivity, the applicable royalty on net sales for such product in the United States will be reduced by 50%. The royalties are also subject to reduction by 50% of certain third-party royalty payments or patent litigation damages or settlements which might be required to be paid by us if litigation were to arise, with any such reductions capped at 50% of the amounts otherwise payable during the applicable royalty payment period.

In addition to the foregoing, we are obligated to pay Infinity an additional royalty of 4% on worldwide net sales of any products in an oncology indication containing duvelisib to cover the reimbursement of research and development costs owed by Infinity to Mundipharma International Corporation Limited (MICL) and Purdue Pharmaceutical Products L.P. (Purdue). Once Infinity has fully reimbursed MICL and Purdue, the royalty obligations will be reduced to 1% of net sales in the United States. These trailing MICL royalties are payable until the later to occur of the last-to-expire of specified patent rights and the expiration of non-patent regulatory exclusivities in a country. Each of the above royalty rates is reduced by 50% on a product-by-product and country-by-country basis if the applicable royalty is payable solely on the basis of non-patent regulatory exclusivity. In addition, the trailing MICL royalties are subject to reduction by 50% of certain third-party royalty payments or patent litigation damages or settlements which might be required to be paid by the us if litigation were to arise, with any such reductions capped at 50% of the amounts otherwise payable during the applicable royalty payment period.

In July 2012, we entered into a license agreement with Pfizer under which Pfizer granted us worldwide, exclusive rights to research, develop, manufacture and commercialize products containing certain of Pfizer's inhibitors of FAK (the FAK Products), including defactinib, 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 FAK 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 FAK 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 \$2.0 million in developmental milestones and up to an additional \$125.0 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 FAK Products. Our royalty obligations with respect to each of the FAK Products in each country begin on the date of first commercial sale of the FAK Products in that country, and end on the later of 10 years after the date of first commercial sale of the FAK Products 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.



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### OFF BALANCE SHEET ARRANGEMENTS

We did not have any off-balance sheet arrangements 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, 2017, we had federal and state net operating loss carryforwards of \$237.0 million and \$235.9 million, respectively, which are available to reduce future taxable income. We also had federal and state tax credits of \$14.1 million and \$1.7 million, respectively, which may be used to offset future tax liabilities. The net operating loss and tax credit carryforwards will expire at various dates through 2037. 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, 2017, we recorded a 100% valuation allowance against our net operating loss and tax credit carryforwards of \$85.8 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

In January 2017, the Financial Accounting Standards Board (FASB) issued Accounting Standard Update (ASU) 2017-03, Accounting Changes and Error Corrections (Topic 250) and Investments – Equity Method and Joint Ventures (Topic 323): Amendments to SEC Paragraphs Pursuant to Staff Announcements at the September 22, 2016 and November 17, 2016 EITF Meetings. ASU 2017-03 clarifies the SEC staff's expectations about the extent of disclosures that a registrant is expected to provide regarding the impact that the adoption of ASUs 2014-09 (Revenue from Contracts with Customers), 2016-02 (Leases) and 2016-13 (Measurement of Credit Losses on Financial Instruments) will have on its financial statements. It also conforms SEC guidance on accounting for tax benefits resulting from investments in affordable housing projects to the guidance in ASU 2014-01, Investments -Equity Method and Joint Ventures (Topic 323). The guidance under this ASU was effective upon issuance and did not have a material impact on our disclosures.

In October 2016, the FASB issued ASU 2016-17, Consolidation (Topic 810): Interests Held through Related Parties That Are under Common Control. ASU 2016-17 updates ASU 2015-02. Under the amendments, a single decision maker is not required to consider indirect interests held through related parties that are under common control with the single decision maker to be the equivalent of direct interests in their entirety. Instead, a single decision maker is required to include those interests on a proportionate basis consistent with indirect interests held through other related parties. ASU 2016-17 was effective for annual and interim periods beginning after December 15, 2016. We adopted this standard effective January 1, 2017. The adoption of this ASU did not have an effect on our consolidated financial statements or disclosures.

In March 2016, the FASB issued ASU 2016-09, Compensation – Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting. ASU 2016-09 simplifies the accounting for share-based compensation arrangements, including the accounting for forfeitures, income tax consequences, classification of awards as either

equity or liabilities and classification on the statement of cash flows. The standard was effective for annual and interim periods beginning after December 15, 2016, with early adoption permitted. We adopted ASU 2016-09 effective January 1, 2017. Upon adoption, we elected to begin accounting for forfeitures as they occur, rather than estimating a forfeiture rate, and recorded an immaterial cumulative-effect adjustment to opening accumulated deficit. Also upon adoption, we recognized all previously unrecognized tax benefits, which would have resulted in the recognition of an immaterial cumulative-effect adjustment to opening accumulated deficit; however, these unrecognized tax benefits were recorded as a deferred tax asset, which was fully offset by a

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valuation allowance. Therefore, the recognition of these benefits had no net cumulative-effect on opening accumulated deficit upon adoption.

### 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 \$86.7 million and \$80.9 million as of December 31, 2017 and 2016, respectively, consisting of cash, U.S. Government money market funds, overnight repurchase agreements collateralized by government agency securities or U.S. Treasury securities, corporate bonds and commercial paper of publicly traded companies. 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, 2017, an immaterial amount of our total liabilities was denominated in currencies other than the functional currency.

As of December 31, 2017, we have borrowed \$15.0 million under the Amended Loan Agreement. The Amended Loan Agreement bears interest per annum equal to the greater of either (a) 10.5% or (b) the lesser of (i) 12.75% and (ii) the sum of (x) 10.5% plus (y) (A) the prime rate minus (B) 4.5%. Changes in interest rates can cause interest charges to fluctuate under the Amended Loan Agreement. A 10% increase in current interest rates would have resulted in an immaterial increase in the amount of cash interest expense for the year ended December 31, 2017.

### 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 26 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

#### Limitations on Effectiveness of Controls and Procedures

In designing and evaluating our disclosure controls and procedures and internal control over financial reporting, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives. In addition, the design of disclosure controls and procedures and internal control over financial reporting must reflect the fact that there are resource constraints and that management is required to apply judgment in evaluating the benefits of possible controls and procedures relative to their costs.

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 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.

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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 U.S. generally accepted accounting principles (GAAP), 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 GAAP, 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 (2013 Framework). Based on this evaluation, our management concluded that our internal control over financial reporting was effective as of December 31, 2017.

The effectiveness of our internal control over financial reporting as of December 31, 2017 has been audited by Ernst & Young LLP, an independent registered public accounting firm, as stated in their report which is included herein.

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, 2017 that has materially affected, or is reasonably likely to materially affect, the Company's internal control over financial reporting.

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### Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of Verastem, Inc.

### Opinion on Internal Control over Financial Reporting

We have audited Verastem, Inc.'s internal control over financial reporting as of December 31, 2017, based on criteria established in Internal Control—Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission 2013 framework (the COSO criteria). In our opinion, Verastem, Inc. (the Company) maintained, in all material respects, effective internal control over financial reporting as of December 31, 2017, based on the COSO criteria.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the 2017 consolidated financial statements of the Company and our report dated March 13, 2018 expressed an unqualified opinion thereon that included an explanatory paragraph regarding the Company's ability to continue as a going concern.

### Basis for Opinion

The Company's management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting included in the accompanying Management's Report on Internal Control Over Financial Reporting. Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects.

Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

### Definition and Limitations of Internal Control Over Financial Reporting

A company's internal control over financial reporting is a process designed 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. A company's internal control over financial reporting 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 the assets of the company; (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 of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

/s/ Ernst & Young LLP

Boston, Massachusetts

March 13, 2018

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Item 9B. Other Information

None.

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## PART III

## ITEM 10. DIRECTORS, EXECUTIVE OFFICERS, AND CORPORATE GOVERNANCE

## DIRECTORS AND EXECUTIVE OFFICERS

The following table sets forth the name, age and position of each of our directors and executive officers as of February 28, 2018.

Name	Age	Position
Robert Forrester	54	President, Chief Executive Officer and Director
Michael Kauffman, M.D., Ph.D.	54	Lead Director
Timothy Barberich	70	Director
Alison Lawton	56	Director
S. Louise Phanstiel	59	Director
Eric Rowinsky, M.D.	61	Director
Brian Stuglik, R.Ph.	58	Director
Bruce Wendel	64	Director
Steven Bloom	57	Chief Strategy Officer
Julie B. Feder	47	Chief Financial Officer
Diep Le, M.D., Ph.D.	50	Chief Medical Officer
Joseph Lobacki	59	Chief Commercial Officer
Daniel Paterson	57	Chief Operating Officer

Robert Forrester, age 54, is a Class III director who has served as a member of our Board of Directors since July 2013. Mr. Forrester has served as our Chief Executive Officer since July 2013, as our Chief Operating Officer from March 2011 until July 2013 and as 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. Prior to his operating roles, Mr. Forrester was a managing director of the Proprietary Investment Group at MeesPierson, part of the Fortis Group, investing in life science companies. Prior to MeesPierson, Mr. Forrester worked for the investment banks, BZW (now Barclays Capital) and UBS, in the corporate finance groups undertaking mergers and acquisitions and public and private financing transactions. Mr. Forrester started his career as a lawyer with Clifford Chance in London and Singapore. He earned his LL.B. from Bristol University in England. The Board of Directors believes that Mr. Forrester's qualifications to sit on the Board include his previous experience serving in leadership positions within the biopharmaceutical industry and his position as our President and Chief Executive Officer.

Michael Kauffman M.D., age 54, is a Class I director who has served as a member of our Board of Directors since November 2012. Dr. Kauffman has been the President and Chief Executive Officer of Karyopharm Therapeutics Inc., a publicly traded biotechnology company, since January 2011 and was a Science Advisor to Bessemer Venture Partners from 2008 to 2011. Dr. Kauffman was the Chief Medical Officer of Onyx Pharmaceuticals, Inc., a publicly traded biotechnology company, from November 2009 until December 2010. Dr. Kauffman was the Chief Medical Officer of Proteolix, Inc., a privately held pharmaceutical company, from April 2009 until November 2009, when it was acquired by Onyx. From September 2002 until July 2008, Dr. Kauffman was the President and Chief Executive Officer of EPIX Pharmaceuticals, Inc., a publicly traded biotechnology company that underwent liquidation

proceedings in 2009. Dr. Kauffman joined Predix Pharmaceuticals, Inc., the predecessor to EPIX, in September 2002, as President and Chief Executive Officer. From 1997 to 2002, he held a number of senior medical and program leadership positions at Millennium Pharmaceuticals, Inc., then a publicly traded biotechnology company, including Vice President, Medicine and VELCADE Program Leader as well as co-founder and Vice President of Medicine at Millennium Predictive Medicine, a wholly-owned subsidiary of Millennium. Dr. Kauffman also served as Medical Director at Biogen Corporation (now Biogen Inc., a publicly traded biotechnology company). Dr. Kauffman has served on the board of directors of Zalicus, Inc., on the board of directors of Karyopharm since January 2011, on the board of directors of Infinity Pharmaceuticals, Inc., and on the

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board of directors of Kezar Life Sciences Inc. Dr. Kauffman received an M.D. and Ph.D. in molecular biology and biochemistry from Johns Hopkins University and holds a B.A. in biochemistry from Amherst College. Dr. Kauffman trained in Internal Medicine at Beth Israel Deaconess and Massachusetts General Hospitals. He is board certified in internal medicine. The Board of Directors believes that Dr. Kauffman's qualifications to sit on the Board include the combination of his significant business, clinical development and leadership experience at public life sciences companies and his medical and scientific background.

Timothy Barberich, age 70, is a Class II director who has served as a member of our Board of Directors since March 2014. Mr. Barberich is founder and former Chairman and Chief Executive Officer of Sepracor Inc., a publicly traded, research-based, pharmaceutical company based in Marlborough, Massachusetts, which was acquired by Dainippon Sumitomo Pharma Co., Ltd. in 2009. He founded Sepracor in 1984 and served as Chief Executive Officer from 1984 to May 2007 and as Chairman of the Board from 1990 to 2009. Mr. Barberich has been Chairman of BioNevia Pharmaceuticals since June 2008 and Chief Executive Officer since 2014. He currently serves on the board of directors of publicly traded GI Dynamics, and on the board of directors of the privately held company, Frequency Therapeutics, Inc. He has also served on the boards of directors of Neurovance Inc. until it was acquired by Otsuka Pharmaceutical Co., Ltd. in 2017, Inotek Pharmaceuticals, Inc., HeartWare, International, Inc., Tokai Pharmaceuticals, BioSphere Medical, Inc. and GeminX Pharmaceuticals. Mr. Barberich has also served on the board of trustees of Boston Medical Center and the board of the Pharmaceutical Research and Manufacturers' Association (PhRMA). Prior to founding Sepracor, Mr. Barberich spent 10 years as a senior executive at Bedford, Massachusetts-based Millipore Corporation. Mr. Barberich is a graduate of Kings College and holds a Bachelor's of Science degree in Chemistry. The Board of Directors believes that Mr. Barberich's qualifications to sit on the Board include his significant experience in the development and commercialization of pharmaceutical products, his leadership experience at other pharmaceutical companies and his service on other boards of directors.

Alison Lawton, age 56, is a Class II director who has served as a member of our Board of Directors since November 2012. Ms. Lawton has been the President and Chief Operating Officer at Kaleidon Biosciences, Inc. since December 2017. Prior to this, Ms. Lawton served as the Chief Operating Officer at Aura Biosciences January 2015 to December 2017, and was Chief Operating Officer of OvaScience, Inc., a publicly traded life sciences company, from January 2013 until December 2013. From 1991 to 2012, Ms. Lawton worked at various positions of increasing responsibility at Genzyme Corporation (Genzyme) and subsequently at Sanofi-Aventis, following its 2011 acquisition of Genzyme, each a global biopharmaceutical company. Ms. Lawton served as head of Genzyme Biosurgery, where she was responsible for Genzyme's global orthopedics, surgical and cell therapy and regenerative medicine businesses. Prior to that, Ms. Lawton oversaw Global Market Access at Genzyme, which included Global Regulatory Affairs, Global Health Outcomes and Strategic Pricing, Global Public Policy, and Global Product Safety & Risk Management. Before joining Genzyme, Ms. Lawton worked for seven years in the United Kingdom at Parke-Davis, a pharmaceutical company. Ms. Lawton serves on the board of directors of ProQR Therapeutics a publicly traded biopharmaceutical companies. She also served on the boards of directors of CoLucid Pharmaceuticals, Inc., until its acquisition by Eli Lilly in 2017, and Cubist Pharmaceuticals for three years until its acquisition by Merck & Co., Inc. in 2015. Ms. Lawton also serves on the Scientific Advisory Board of the private biotechnology company X4 Pharmaceuticals. She is a former President and Chair of the Board of Regulatory Affairs Professional Society and former FDA Advisory Committee member for Cell and Gene Therapy Committee. The Board of Directors believes that Ms. Lawton's qualifications to sit on the Board include significant operational, international, regulatory and senior management experience within the pharmaceutical and biotechnology industries and her experience serving on boards of directors within the industry.

S. Louise Phanstiel, age 59, is a Class I director who has served as a member of our Board of Directors since September 2012. Ms. Phanstiel held several important positions at WellPoint, Inc. from 1996 to 2007, including President, Specialty Products (2003 to 2007), Senior Vice President, Chief of Staff and Corporate Planning in the Office of the Chairman (2000 to 2003), and Senior Vice President, Chief Accounting Officer, Controller, and Chief

Financial Officer for all WellPoint, Inc. subsidiaries, including Blue Cross of California (1996 to 2000). Previously, Ms. Phanstiel was a partner at the international services firm of Coopers & Lybrand, where she served clients in life and property/casualty insurance, high technology, and higher education. Ms. Phanstiel has served on the board of directors of Myriad Genetics since September 2009, and formerly served on the boards of directors of Inveresk Research Group, Inc. and Charles River Laboratories, Inc. Ms. Phanstiel received a B.A. degree in Accounting from Golden Gate University and is a Certified Public Accountant. The Board of Directors

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believes that Ms. Phanstiel's qualifications to sit on the Board include her significant financial, investment, and management expertise, and her experience managing and serving as a director of publicly traded companies.

Eric Rowinsky, age 61, is a Class I director who has served as a member of our Board of Directors since May 2017. Dr. Rowinsky has been the Executive Director and President of RGenix, Inc., since June 2016. He also has served as the Chief Scientific Officer of Clearpath Development Co. since June 2016. Prior to this, Dr. Rowinsky served as the Head of Research and Development, Chief Medical Officer and Executive Vice President of Stemline Therapeutics, Inc. from February 2011 to January 2016. In 2010, Dr. Rowinsky co-founded Primrose Therapeutics and became its Chief Executive Officer until it was acquired in 2011. From 2005 to 2010, he served as the Chief Medical Officer and Executive Vice President of Clinical Development and Regulatory Affairs of ImClone Systems Incorporated, a life sciences company focused on monoclonal antibodies, which was acquired by Eli Lilly. Previous to that, Dr. Rowinsky held several positions at the Cancer Therapy and Research Center's Institute of Drug Development, including Director of the Institute and SBC Endowed Chair for Early Drug Development. Prior to that, he served as Clinical Professor of Medicine in the Division of Medical Oncology at the University of Texas Health Science Center at San Antonio and as Associate Professor of Oncology at the Johns Hopkins University School of Medicine. Dr. Rowinsky has served on the boards of directors of Biogen Idec, Inc., Navidea, and Fortress Biosciences, Inc., all public life sciences companies since 2010, and formerly served on the board of directors of BIND Therapeutics, a life-science company acquired by Pfizer. Dr. Rowinsky received a B.A. degree in Liberal Arts from New York University and earned his M.D. from Vanderbilt University. The Board of Directors believes that Dr. Rowinsky's qualifications to sit on the Board include his extensive research and drug development experience, oncology expertise, corporate strategy, and broad scientific and medical knowledge.

Brian Stuglik R.Ph., age 58, is a Class II director who has served as a member of our Board of Directors since September 2017. Mr. Stuglik founded Proventus Health Solutions in January 2016 and has over three decades of experience in U.S. and international pharmaceutical development, product strategy, and commercialization. Prior to founding Proventus Health Solutions, Mr. Stuglik served as the Vice President and Chief Marketing Officer for the Oncology division of Eli Lilly and Company from 2009 to December 2015. Mr. Stuglik received a Bachelor of Science in Pharmacy from Purdue University and holds memberships in the American Society of Clinical Oncology, the American Association of Cancer Research, and the International Association for the Study of Lung Cancer. The Board of Directors believes that Mr. Stuglik's qualifications to sit on the Board include his extensive experience in pharmaceutical development, product strategy and commercialization.

Bruce Wendel, age 64, is a Class III director who has served as a member of our Board of Directors since June 2016. Mr. Wendel has been Chief Strategic Officer of Hepalink USA, the U.S. subsidiary of Shenzhen Hepalink Pharmaceutical Company, since June 2012. Prior to this, Mr. Wendel served as Vice Chairman and Chief Executive Officer of Abraxis BioScience, LLC, from January 2010 to December 2010, where he oversaw the development and commercialization of Abraxane®. He also led the negotiations that culminated in the acquisition of Abraxis by Celgene in a deal valued at over \$2.9 billion. Prior to Abraxis, Mr. Wendel served in business and corporate development roles of increasing responsibility at American Pharmaceutical Partners, IVAX Corporation and Bristol-Myers Squibb. Mr. Wendel currently serves on the board of directors of ProMetic Life Sciences, Inc., a publicly traded biopharmaceutical company. Mr. Wendel earned a juris doctorate degree from Georgetown University Law School, and a B.S. from Cornell University. The Board of Directors believes that Mr. Wendel's qualifications to sit on the Board include his experience building companies and bringing oncology drugs to the market, his oversight of the development and commercialization of Abraxane®, and his life sciences industry experience and knowledge.

Steven Bloom, age 57, has served as our Chief Strategy Officer since December 2017, our Senior Vice President of Corporate Development from January 2017 to November 2017 and as our Vice President of Commercial Planning and External Affairs from January 2015 until January 2017. Prior to joining us in March 2014, Mr. Bloom served as Senior Vice President at Ziopharm Oncology from March 2008 to March 2014. Before joining Ziopharm, Mr. Bloom

was Vice President for the health informatics company Pharmedics and spent the first 19 years of his career at Eli Lilly and Company in leadership roles in marketing, sales and corporate affairs.

Julie B. Feder, age 47, has served as our Chief Financial Officer since July 2017. Prior to joining us, Ms. Feder served as the Chief Financial Officer for the Clinton Health Access Initiative (CHAI) from September 2011 to July 2017. Prior to joining CHAI, Ms. Feder spent three years at Genzyme Corporation, first as Vice President of

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Internal Audit and also as Finance Integration Leader. In these roles, she managed the day-to-day operations of Genzyme's global internal audit function, while leading the Genzyme Global Finance integration into Sanofi's organization following Sanofi's acquisition of Genzyme.

Diep Le, M.D., Ph.D., age 50, has served as our Chief Medical Officer since October 2017. Prior to joining us, Dr. Le served as the Vice President, Immuno-Oncology Innovative Medicines at MedImmune (a subsidiary of AstraZeneca) from October 2015 to June 2017 and led the product development teams for multiple high-priority immuno-oncology assets. Prior to that, Dr. Le served as the Executive Director and Global Clinical Program Lead at Novartis Oncology from October 2013 to October 2015, and various roles of increasing responsibility at GlaxoSmithKline from June 2009 to October 2013, where she led the MEK inhibitor, trametinib (Mekinist™), from the first-in-human studies to FDA approval.

Joseph Lobacki, age 59, has served as our Chief Commercial Officer since January 2018. Prior to joining us, Mr. Lobacki served as the Chief Operating Officer of Finch Therapeutics Group from November 2016 to December 2017, the Chief Commercial Officer and Executive Council Member of Medivation, Inc. from December 2014 to October 2016, and as the General Manager of Oncology at Idera Pharmaceuticals from April 2014 to December 2014. Prior to that Mr. Lobacki served as a commercial and business operations consultant for biotechnology companies from June 2012 to April 2014 and as the Senior Vice President and Chief Commercial Officer of Micromet Inc., where he oversaw commercial activities including medical affairs and strategic marketing.

Daniel Paterson, age 57, 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 in March 2012, Mr. Paterson was a consultant in 2011. From 2009 through 2010, Mr. Paterson was the Chief Operating Officer of On-Q-ity. Mr. Paterson was the President and Chief Executive Officer 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.

### Section 16(a) Beneficial Ownership Reporting Compliance

Our directors, executive officers and beneficial owners of more than 10% of our common stock are required under Section 16(a) of the Securities Exchange Act of 1934, as amended, to file reports of ownership and changes in ownership of our securities with the Securities and Exchange Commission (SEC). We believe that, during the year ended December 31, 2017, our directors, executive officers and beneficial owners of more than 10% of the Company's common stock complied with all Section 16(a) filing requirements.

### Code of Business Conduct and Ethics

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

### Board Committees

Our board of directors has established an audit committee, a nominating and corporate governance committee, and a compensation committee, each of which operates under a charter that has been approved by our board. Our board of directors has determined that all of the members of the audit committee, the compensation committee and the

nominating and corporate governance committee are independent as defined under Nasdaq Marketplace Rules, including, in the case of all the members of our audit committee, the independence requirements contemplated by Rule 10A-3 under the Securities Exchange Act of 1934. No changes have been made to the procedures by which our stockholders may recommend nominees to our board of directors.



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## Audit committee

The members of our audit committee are Louise Phanstiel, Timothy Barberich, and Michael Kauffman. Our board of directors has determined that Louise Phanstiel is an “audit committee financial expert” as defined in the applicable SEC rules.

## Nominating and corporate governance committee

The members of our nominating and corporate governance committee are Timothy Barberich, Eric Rowinsky and Bruce Wendel.

## Compensation committee

The members of our compensation committee are Alison Lawton, Michael Kauffman, and Brian Stuglik.

## ITEM 11. EXECUTIVE COMPENSATION

## NAMED EXECUTIVE OFFICER COMPENSATION,

## COMPENSATION DISCUSSION AND ANALYSIS

Our named executive officers for the fiscal year ended December 31, 2017 were:

- Robert Forrester, our President and Chief Executive Officer;
- Julie B. Feder, our Chief Financial Officer; and
- Diep Le, M.D., Ph.D., our Chief Medical Officer.

## Summary Compensation Table

The following table provides information regarding the total compensation for services rendered in all capacities that was earned during the fiscal year indicated by our named executive officers.

Name and Principal Position	Year	Salary (\$)	Bonus (\$)	Option Awards (\$)(1)	Non-Equity Incentive Plans (\$)(2)	All Other Compensation (\$)(3)	Total (\$)
Robert Forrester	2017	535,000	—	294,804	321,000	15,014	1,165,818
Chief Executive Officer	2016	525,000	—	245,844	321,000	12,690	1,104,534

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Julie B. Feder (4) Chief Financial Officer	2017	150,385	—	878,024	60,000	6,708	1,095,117
Diep Le, M.D. Ph.D. (5) Chief Medical Officer	2017	76,923	95,000	(6) 1,125,863	40,000	3,365	1,341,151

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- (1) The amounts reflect the aggregate grant date fair value of option awards granted during the year computed in accordance with the provisions of Financial Accounting Standards Board Accounting Standard Codification Topic 718 (FASB ASC Topic 718). For information regarding assumptions underlying the value of stock awards, see Note 7 to our financial statements and the discussion under Part II, Item 7 “Management’s Discussion and Analysis of Financial Condition and Results of Operations—Critical Accounting Policies—Stock-Based Compensation,” of this Annual Report on Form 10-K for the year ended December 31, 2017.
- (2) The amounts shown for non-equity incentive plan compensation represent amounts earned for the fiscal years ended December 31, 2017 and 2016. Amounts earned for 2017 were paid in 2018, and amounts earned in 2016 were paid in 2017.

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- (3) The amounts shown represent the sum of 401(k) contributions, Health Savings Account contributions, and the dollar value of life insurance premiums paid by the Company for the applicable named executive officer.
- (4) Ms. Feder has served as our Chief Financial Officer since July 10, 2017.
- (5) Dr. Le has served as our Chief Medical Officer since October 9, 2017.
- (6) The amount reflects a one-time sign-on bonus of \$95,000 paid to Dr. Le.

Narrative Discussion of Summary Compensation Table

Employment Agreements

We have entered into an employment agreement with each of our named executive officers. Each of the employment agreements provides that employment will continue for an indefinite period until either the Company or the employee provides written notice of termination in accordance with the terms of the agreement.

Robert Forrester

Pursuant to his amended and restated employment agreement, as of July 1, 2013, Mr. Forrester was entitled to an initial base salary of \$490,000, subject to increase from time to time by the Board of Directors. As of January 1, 2018, Mr. Forrester's annual base salary is \$555,000. Mr. Forrester is eligible to receive a bonus of 60% of his current annual base salary. Subject to Mr. Forrester's execution of an effective release of claims, Mr. Forrester would be entitled to the severance payments described below if we terminate his employment without cause, as defined in his employment agreement, or if Mr. Forrester terminates his employment for good reason, as defined in his employment agreement.

If Mr. Forrester's employment is terminated by us without cause or by Mr. Forrester for good reason, absent a change in control, as defined in his employment agreement, we would be obligated, (1) to pay Mr. Forrester his base salary for a period of 12 months following the termination of his employment, (2) to accelerate the vesting of the portion of any equity awards granted prior to the date of his amended and restated employment agreement that, by their terms, vest only based on the passage of time and that would have vested during the 12-month period following the termination of his employment, (3) to pay Mr. Forrester any bonus which has been awarded, but not yet paid on the date of termination and (4) if Mr. Forrester exercises his right to continue participation in our health and dental plans under the federal law known as COBRA, to pay Mr. Forrester a monthly cash amount equal to the full premium cost of that participation for 12 months following such termination of employment (or, if earlier, until the time when Mr. Forrester becomes eligible to enroll in the health or dental plan of a new employer).

If Mr. Forrester's employment is terminated by us without cause or by Mr. Forrester for good reason, in each case within 90 days prior to, or within one year following, a change in control, we would be obligated (1) to pay Mr. Forrester a lump sum amount equal to two times the sum of his then-current annual base salary plus an amount equal to his target bonus, (2) to accelerate the vesting of all outstanding equity awards that, by their terms, vest only based on the passage of time, (3) to pay Mr. Forrester any bonus which has been awarded, but not yet paid on the date of termination and (4) if Mr. Forrester exercises his right to continue participation in our health and dental plans under the federal law known as COBRA, to pay Mr. Forrester a monthly cash amount equal to the full premium cost of that participation for 24 months following such termination of employment (or, if earlier, until the time when provided that such benefits shall end when Mr. Forrester becomes eligible to enroll in the health or dental plan of a new employer).

To the extent that any severance or compensation payable to Mr. Forrester pursuant to his employment agreement or otherwise in connection with a change in control of the Company would be subject to the excise tax imposed under Section 4999 of the Internal Revenue Code, Mr. Forrester would be entitled to an additional cash payment equal to an amount calculated by multiplying the grossed-up amount of such payments (i.e., an amount such that net amount retained by Mr. Forrester after payment of all applicable taxes, interest and penalties thereon is equal to the total payments payable to him) by a fraction, the numerator of which is the portion of such payments



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related to equity awards granted prior to the execution of his employment agreement and the denominator of which is the portion of such payments related to all equity awards granted to him. However, if it would result in a greater amount payable to Mr. Forrester, Mr. Forrester would instead be entitled to either the full amount of the total payments payable in connection with a change in control or a reduced amount of the total payments payable in connection with a change in control, whichever results in the greater economic benefit for Mr. Forrester.

Julie B. Feder

Pursuant to her employment agreement, Ms. Feder was entitled to an initial base salary of \$340,000, subject to increase from time to time by the Board of Directors. As of January 1, 2018, Ms. Feder's annual base salary is \$355,000. Ms. Feder is also eligible to receive a bonus of 35% of her current annual base salary. Subject to Ms. Feder's execution of an effective release of claims, Ms. Feder would be entitled to the severance payments described below if we terminate her employment without cause, as defined in her employment agreement, or if Ms. Feder terminates her employment for good reason, as defined in her employment agreement.

If Ms. Feder's employment is terminated by us without cause or by Ms. Feder for good reason, absent a change in control, as defined in her employment agreement, we would be obligated (1) to pay Ms. Feder her base salary for a period of nine months following such termination of employment, (2) to pay Ms. Feder any bonus which has been awarded, but not yet paid on the date of termination and (3) if Ms. Feder exercises her right to continue participation in our health and dental plans under the federal law known as COBRA, to pay Ms. Feder a monthly cash amount equal to the full premium cost of that participation for nine months following such termination of employment (or, if earlier, until the time when Ms. Feder becomes eligible to enroll in the health or dental plan of a new employer).

If Ms. Feder's employment is terminated by us without cause or by Ms. Feder for good reason, in each case within 90 days prior to, or within 18 months following, a change in control, we would be obligated (1) to pay Ms. Feder a lump sum amount equal to 12 months of her then-current annual base salary, (2) to accelerate the vesting of all outstanding equity awards that, by their terms, vest only based on the passage of time, (3) if Ms. Feder exercises her right to continue participation in our health and dental plans under the federal law known as COBRA, to pay Ms. Feder a monthly cash amount equal to the full premium cost of that participation for 12 months following such termination of employment (or, if earlier, until the time when Ms. Feder becomes eligible to enroll in the health or dental plan of a new employer) and (4) to pay any bonus which has been awarded, but not yet paid on the date of termination.

Diep Le, M.D., Ph.D.

Pursuant to her employment agreement, Dr. Le was entitled to an initial base salary of \$400,000, subject to increase from time to time by the Board of Directors, and a one-time sign-on bonus of \$95,000 that will be earned on the second anniversary of her hire date, but was paid during 2017. If Dr. Le resigns before the second anniversary of her hire date, she must repay the sign-on bonus in full. Dr. Le is also entitled to payment or reimbursement of moving expenses up to \$50,000 associated with relocating to the Boston area, and for reasonable and customary commuting expenses prior to such relocation. As of January 1, 2018, Dr. Le's annual base salary is \$400,000. Dr. Le is also eligible to receive a bonus of 40% of her current annual base salary. Subject to Dr. Le's execution of an effective release of claims, Dr. Le would be entitled to the severance payments described below if we terminate her employment without cause, as defined in her employment agreement, or if Dr. Le terminates her employment for good reason, as defined in her employment agreement.

If Dr. Le's employment is terminated by us without cause or by Dr. Le for good reason, absent a change in control, as defined in her employment agreement, we would be obligated (1) to pay Dr. Le her base salary for a period of nine months following such termination of employment, or if the termination occurs prior to Dr. Le's relocation to Massachusetts, her then-current annual base salary for a period of one month for each full month that has elapsed

between the effective date of her employment agreement and the termination date, up to a maximum of nine months, (2) payment of bonus which has been awarded, but not yet paid on the date of termination and (3) if Dr. Le exercises her right to continue participation in our health and dental plans under the federal law known as COBRA, to pay Dr. Le a monthly cash amount equal to the full premium cost of that participation for a period commensurate with the period over which Dr. Le is entitled to receive salary payments following such termination

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(or, if earlier, until the time when Dr. Le becomes eligible to enroll in the health or dental plan of a new employer).

If Dr. Le's employment is terminated by us without cause or by Dr. Le for good reason, in each case within 90 days prior to, or within 18 months following, a change in control, we would be obligated (1) to pay Dr. Le a lump sum amount equal to 12 months of her then-current annual base salary, (2) to accelerate the vesting of all outstanding equity awards that, by their terms, vest only based on the passage of time, (3) if Dr. Le exercises her right to continue participation in our health and dental plans under the federal law known as COBRA, to pay Dr. Le a monthly cash amount equal to the full premium cost of that participation for 12 months following such termination of employment (or, if earlier, until the time when Dr. Le becomes eligible to enroll in the health or dental plan of a new employer) and (4) to pay any bonus which has been awarded, but not yet paid on the date of termination.

## Pension Benefits and Deferred Compensation

We maintain a defined contribution employee retirement plan for our employees. Our 401(k) plan is intended to qualify as a tax-qualified plan under Section 401(a) of the Internal Revenue Code. Employee contributions may be made on a pre-tax basis or after-tax (Roth) basis. The 401(k) plan provides for employer matching contributions equal to (1) 100% of employee deferral contributions up to a deferral rate of 3% of eligible compensation plus (2) 50% of employee deferral contributions up to a deferral rate of an additional 2% of eligible compensation.

## Outstanding Equity Awards at Fiscal Year-End

The following table provides information regarding equity awards held by each of our named executive officers that were outstanding as of December 31, 2017.

Name	Option Awards		Option Exercise Price (\$)	Option Expiration Date
	Number of Securities Underlying Unexercised Options Exercisable (#)	Number of Securities Underlying Unexercised Options Unexercisable (#)		
Robert Forrester	250,000	—	(1) 9.85	1/15/2023
	50,000	—	(2) 14.18	9/17/2023
	250,000	—	(3) 13.59	1/7/2024
	250,000	—	(4) 13.59	1/7/2024
	185,963	84,525	(5) 9.19	1/8/2025
	268,000	—	(6) 2.13	11/8/2025
	132,000	—	(7) 1.86	1/1/2026
	100,000	—	(8) 1.37	6/14/2026
	—	360,000	(9) 1.20	1/9/2027
Julie B. Feder	—	370,000	(10) 3.45	7/10/2027
Diep Le, M.D.	—	300,000	(11) 4.63	10/9/2027
	—	70,000	(12) 4.63	10/9/2027

(1)

This option was granted on January 15, 2013. The option vested as to 25% of the shares underlying the option on the first anniversary of the grant date and, thereafter, as to 6.25% of the shares underlying the option at the end of each successive three-month period following the first anniversary of the grant date until the fourth anniversary of the grant date.

- (2) This option was granted on September 17, 2013. The option vested as to 6.25% of the shares underlying the option on October 1, 2013 and, thereafter, as to 6.25% of the shares underlying the option at the end of each successive three-month period until July 1, 2017.
- (3) This option was granted on January 7, 2014. The option vested as to 25% of the shares underlying the option on



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July 1, 2014 and, thereafter, as to 6.25% of the shares underlying the option on the last day of each calendar quarter after such date, through June 30, 2017.

- (4) This option was granted on January 7, 2014. The option vested as to 25% of the shares underlying the option on the first anniversary of the grant date and, thereafter, as to 6.25% of the shares underlying the option on the last day of each calendar quarter after such date, through December 31, 2017.
- (5) This option was granted on January 8, 2015. The option vests as to 25% of the shares underlying the option on the first anniversary of the grant date and, thereafter, as to 6.25% of the shares underlying the option at the end of each successive three-month period following the first anniversary of the grant date until the fourth anniversary of the grant date.
- (6) This option was granted on November 9, 2015. The option vested as to 50% of the shares underlying the option on the first anniversary of the grant date and, thereafter, as to the remaining 50% of the shares underlying the option on the second anniversary of the grant date.
- (7) This option was granted on January 1, 2016. The option vested as to 50% of the shares underlying the option on November 9, 2016 and, thereafter, as to the remaining 50% of the shares underlying the option on November 9, 2017.
- (8) This option was granted on June 14, 2016. The option vested as to 50% of the shares underlying the option upon satisfaction of a certain performance milestone by June 2017, and as to the remaining 50% of the shares underlying the option upon satisfaction of a certain performance milestone in September 2017.
- (9) This option was granted on January 9, 2017. The option vests as to 25% of the shares underlying the option on the first anniversary of the grant date and, thereafter, as to 6.25% of the shares underlying the option at the end of each successive three-month period following the first anniversary of the grant date until the fourth anniversary of the grant date.
  - (10) This option was granted on July 10, 2017. The option vests as to 25% of the shares underlying the option on the first anniversary of the grant date and, thereafter, as to 6.25% of the shares underlying the option at the end of each successive three-month period following the first anniversary of the grant date until the fourth anniversary of the grant date.
- (11) This option was granted on October 9, 2017. The option vests as to 25% of the shares underlying the option on the first anniversary of the grant date and, thereafter, as to 6.25% of the shares underlying the option at the end of each successive three-month period following the first anniversary of the grant date until the fourth anniversary of the grant date.
- (12) This option was granted on October 9, 2017. The option vests as to 100% of the shares underlying the option upon satisfaction of a certain performance milestone.

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## DIRECTOR COMPENSATION

## Director Compensation

The following table summarizes the compensation paid to or earned by our directors during the year ended December 31, 2017:

Name	Fees Earned	Option	Total (\$)
	or Paid in Cash (\$)	Awards (\$)(1)(2)	
Timothy Barberich	53,000	35,742	88,742
Paul Friedman, M.D. (3)	16,125	—	16,125
Michael Kauffman, M.D., Ph.D.	79,000	35,742	114,742
Alison Lawton	55,000	35,742	90,742
S. Louise Phanstiel	60,000	35,742	95,742
Eric Rowinsky, M.D.	33,125	71,483	104,608
Brian Stuglik, R.Ph.	14,831	168,776	183,607
Bruce Wendel	49,320	35,742	85,062

- (1) Amounts shown represent the aggregate grant date fair value of stock option awards granted to the director and calculated in accordance with FASB ASC Topic 718. For information regarding assumptions underlying the value of stock awards, see Note 7 to our financial statements and the discussion under Part II, Item 7 “Management’s Discussion and Analysis of Financial Condition and Results of Operations—Critical Accounting Policies—Stock-Based Compensation,” of this Annual Report on Form 10-K for the year ended December 31, 2017.
- (2) The number of stock options awarded to any non-employee director who received a grant during 2017 was 25,000, with the exception of Dr. Rowinsky and Mr. Stuglik who each received 50,000 stock options as a result of their new appointments to our Board of Directors
- (3) Dr. Friedman resigned from our Board of Directors effective April 27, 2017.

The following table sets forth, as of December 31, 2017, the aggregate number of exercisable and unexercisable stock option awards held by our directors:

Name	Option Awards		
	Exercisable (#)	Unexercisable (#)	Total (#)
Timothy Barberich	110,099	12,498	122,597
Michael Kauffman, M.D., Ph.D.	124,478	12,498	136,976
Alison Lawton	124,478	12,498	136,976
S. Louise Phanstiel	126,841	12,498	139,339
Eric Rowinsky, M.D.	25,002	24,998	50,000
Brian Stuglik, R.Ph.	12,501	37,499	50,000
Bruce Wendel	62,502	12,498	75,000

## Non-Employee Director Compensation

Under our non-employee director compensation policy, each non-employee director receives an annual base retainer of \$40,000. In addition, our non-employee directors receive the following cash compensation for Board services, as applicable:

- the non-executive Lead Director of the Board of Directors receives an additional annual retainer of \$25,000;
- each chairperson of our Audit, Compensation and Nominating and Corporate Governance Committees receives an additional annual retainer of \$20,000, \$15,000 and \$10,000, respectively; and

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· each member of our Audit, Compensation and Nominating and Corporate Governance Committees receives an additional retainer of \$8,000, \$6,000 and \$5,000, respectively.  
All amounts are paid in quarterly installments.

In addition, our non-employee directors receive stock options as compensation for their service on our Board of Directors. Newly appointed non-employee directors receive a one-time initial award of options to purchase 50,000 shares of our common stock, which vest monthly over a one-year period subject to the director's continued service on the Board of Directors. Thereafter, each non-employee director who was serving on the Board of Directors as of the prior year's annual meeting of the Company's shareholders, receives an annual award of options to purchase shares of our common stock, which vest monthly over a one-year period, subject to the director's continued service on the Board of Directors (Annual Grant). Additionally, each non-employee director who has served 12 months on the Board of Directors as of the date of the annual meeting of the Company's shareholders, but has not yet received an Annual Grant also receives a pro-rated grant (based on the Annual Grant for such year) to reflect the time such director has served on the Board of Directors since the 12-month anniversary of the commencement of such director's service, which vests monthly over a one-year period, subject to the director's continued service on the Board of Directors. In 2017, the Annual Grant consisted of options to purchase 25,000 shares of our common stock.

Mr. Forrester, our President and Chief Executive Officer, does not receive compensation for his service as a director. Mr. Forrester's compensation is described under the heading "Executive Compensation."

## ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT AND RELATED STOCKHOLDER MATTERS

### Equity Compensation Plan Information

The following table contains information about our equity compensation plans as of December 31, 2017.

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)	7,609,728	\$ 5.40	654,630	(3)
Equity compensation plans not approved by security holders(2)	1,110,250	\$ 3.75	2,506,000	

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

(2) In December 2014, 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. As of December 31, 2017, 1,324,000 shares had been granted, 138,750 shares had been exercised and 75,000 shares had been cancelled under this program. In December 2017, the Board of Directors authorized and reserved 2,500,000 additional 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.

- (3) Does not include 1,285,714 shares added to the 2012 Incentive Plan under the evergreen provision on January 1, 2018.

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## SECURITY OWNERSHIP OF

## CERTAIN BENEFICIAL OWNERS, DIRECTORS AND MANAGEMENT

The following table sets forth certain information as of February 28, 2018 (unless otherwise specified), with respect to the beneficial ownership of our common stock by each person who is known to own beneficially more than 5% of the outstanding shares of common stock, each person currently serving as a director, each nominee for director, each named executive officer (as set forth in the Summary Compensation Table above), and all directors and executive officers as a group.

Shares of common stock subject to options, RSUs or other rights to purchase which are now exercisable or are exercisable within 60 days after February 28, 2018 are to be considered outstanding for purposes of computing the percentage ownership of the persons holding these options or other rights but are not to be considered outstanding for the purpose of computing the percentage ownership of any other person. As of February 28, 2018, there were 50,800,908 shares of common stock outstanding.

Name and address of beneficial owner	Number of shares beneficially owned	Percentage of shares beneficially owned	
5% stockholders:			
BVF, Inc. (1) 1 Sansome Street, 30th Floor San Francisco, CA 94104	2,737,000	5.39	%
Directors and Executive Officers			
Robert Forrester (2)	1,851,007	3.53	%
Julie B. Feder	—	—	
Diep Le, M.D.	—	—	
Timothy Barberich (3)	248,431	*	
Michael Kauffman, M.D., Ph.D. (4)	132,810	*	
Alison Lawton (5)	135,310	*	
S. Louise Phanstiel (6)	161,673	*	
Eric Rowinsky, M.D. (7)	41,668	*	
Brian Stuglik, R. Ph. (8)	29,169	*	
Bruce Wendel (9)	70,834	*	
All executive officers and directors as a group (Thirteen persons) (10)	3,822,398	7.06	%

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

(1) Information is based on a Schedule 13G filed with the SEC on January 26, 2018 by Biotechnology Value Fund, L.P. (BVF), Biotechnology Fund II, L.P. (BVF2), Biotechnology Value Trading Fund OS LP (Trading Fund OS), BVF Partners OS Ltd. (Partners OS), BVF Partners LP (Partners), BVF Inc. and Mark N. Lampert (Mr. Lampert), reporting as of January 16, 2018. According to the Schedule 13G, (i) BVF beneficially owns 1,293,127 shares of common stock, (ii) BVF2 beneficially owns 863,522 shares of common stock, and (iii) Trading Fund OS beneficially owns 221,646 shares of common stock. Partner OS, as general partner of Trading Fund OF, may be

deemed to beneficially own the 221,646 shares owned by Trading Fund OS. Partners, as the general partner of BVF, BVF2, the investment manager of Trading Fund OS, and the sole member of Partner OS, may be deemed to beneficially own the 2,737,000 shares of common stock owned by aggregate by BVF, BVF2, Trading Fund OS, and certain Partners management account, including 358,705 shares of common stock owned. BVF Inc., as the general partner of Partners, and Mr. Lampert as a director and officer of BVF Inc. may be deemed to beneficially own the 2,737,000 shares of common stock owned by Partners. The address for these entities is listed in the Schedule 13G as 1 Sansome Street, 30th Floor, San Francisco, CA 94104.

- (2) Consists of 9,000 shares of common stock held by the Claudia Forrester 2001 Trust, 9,000 shares of common stock held by the Iona Forrester 2001 Trust and 200,734 shares of common stock held by Mr. Forrester and 1,632,273 shares of common stock issuable upon the exercise of stock options within 60 days of February 28,

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2018.

- (3) Consists of 130,000 shares of common stock held by Mr. Barberich and 118,431 shares of common stock issuable upon the exercise of stock options within 60 days of February 28, 2018.
- (4) Consists of 132,810 shares of common stock issuable upon the exercise of stock options within 60 days of February 28, 2018.
- (5) Consists of 2,500 shares of common stock held by Ms. Lawton and 132,810 shares of common stock issuable upon the exercise of stock options within 60 days of February 28, 2018.
- (6) Consists of 26,500 shares of common stock held by The Phanstiel Trust and 135,173 shares of common stock issuable upon the exercise of stock options within 60 days of February 28, 2018.
- (7) Consists of 41,668 shares of common stock issuable upon the exercise of stock options within 60 days of February 28, 2018.
- (8) Consists of 29,169 shares of common stock issuable upon the exercise of stock options within 60 days of February 28, 2018.
- (9) Consists of 70,834 shares of common stock issuable upon the exercise of stock options within 60 days of February 28, 2018.
- (10) Includes shares of common stock issuable upon exercise of stock options within 60 days of February 28, 2018.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE

Policies and Procedures for Related Person Transactions

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

Transactions with related persons

If a related person proposes to enter into such a transaction, arrangement or relationship, which we refer to as a “related person transaction,” the related person must report the proposed related person transaction to our principal financial officer. The policy calls for the proposed related person transaction to be reviewed and, if deemed appropriate, approved by our Audit Committee. Whenever practicable, the reporting, review and approval will occur prior to entry into the transaction. If advance review and approval is not practicable, the Audit Committee will review, and, in its discretion, may ratify the related person transaction. The policy also permits the chairman of the Audit Committee to review and, if deemed appropriate, approve proposed related person transactions that arise between Audit Committee meetings, subject to ratification by the Audit Committee at its next meeting. Any related person transactions that are ongoing in nature will be reviewed annually.

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

- the related person’s interest in the related person transaction;
- the approximate dollar value of the amount involved in the related person transaction;



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

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

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

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

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

## Director Independence

As required by the listing standards of The Nasdaq Global Market (Nasdaq), the Board of Directors has affirmatively determined, upon the recommendation of the Nominating and Corporate Governance Committee, that each of our directors and nominees for director other than Robert Forrester, our President and Chief Executive Officer, is independent. To make this determination, our Board of Directors reviews all relevant transactions or relationships between each director and Verastem, its senior management and its independent registered public accounting firm. During this review, the Board considers whether there are any transactions or relationships between directors or any member of their immediate family (or any entity of which a director or an immediate family member is an executive officer, general partner or significant equity holder) and members of our senior management or their affiliates. The Board consults with Verastem's outside corporate counsel to ensure that the Board's determinations are consistent with all relevant securities and other laws and regulations regarding the definition of "independent," including those set forth in pertinent Nasdaq listing standards, as in effect from time to time.

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## ITEM 14. PRINCIPAL ACCOUNTANT FEES AND SERVICES

## Principal Accountant Fees and Services

We regularly review the services and fees of our independent accountants. These services and fees are also reviewed by the Audit Committee on an annual basis. The aggregate fees billed and accrued for the fiscal years ended December 31, 2017 and 2016 for each of the following categories of services are as follows:

Fee Category	2017 (\$)	2016 (\$)
Audit Fees	1,086,000	417,500
Audit-Related Fees	—	—
Tax Fees	—	—
All Other Fees	—	—
Total Fees	1,086,000	417,500

**Audit Fees.** Consist of fees billed and accrued for professional services rendered for the audit of our annual financial statements, the review of interim financial statements and services provided in connection with our registration statements.

**Audit-Related Fees.** Consist of fees billed for assurance and related services that are reasonably related to the performance of the audit or review of our financial statements and are not reported under “Audit Fees.”

**Tax Fees.** Consist of fees billed for tax compliance, tax advice and tax planning and includes fees for tax return preparation.

**All Other Fees.** Consist of fees billed for products and services, other than those described above under Audit Fees, Audit-Related Fees and Tax Fees.

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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.

Item 16. Form 10-K Summary

None.

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EXHIBIT INDEX

Exhibit number	Description of exhibit
3.1	<u>Restated Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.1 to the Annual Report on Form 10 K filed by the Registrant on March 30, 2012)</u>
3.2	<u>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)</u>
4.1	<u>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)</u>
10.1#	<u>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)</u>
10.2*#	<u>Amended and Restated 2012 Incentive Plan</u>
10.3#	<u>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)</u>
10.4*#	<u>Form of Incentive Stock Option Agreement under Amended and Restated 2012 Incentive Plan</u>
10.5#	<u>Form of Nonstatutory 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)</u>
10.6*#	<u>Form of Nonstatutory Stock Option Agreement under Amended and Restated 2012 Incentive Plan</u>
10.7#	<u>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)</u>
10.8#	<u>Amendment to Form of Restricted Stock Unit Agreement under 2012 Incentive Plan (incorporated by reference to Exhibit 10.25 to the Annual Report on Form 10 K filed by the Registrant on March 26, 2013)</u>
10.9*#	<u>Form of Restricted Stock Unit Agreement under Amended and Restated 2012 Incentive Plan</u>
10.10#	<u>Form of Inducement Award Nonstatutory Stock Option Agreement (incorporated by reference to Exhibit 4.4 to the Registration Statement on Form S-8 filed by the Registrant with the Securities and Exchange Commission on December 19, 2014)</u>
10.11*#	<u>Form of Inducement Award Nonstatutory Stock Option Agreement</u>
10.12#	

Amended and Restated Employment Agreement between the Registrant and Robert Forrester, dated January 13, 2012 (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)

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- 10.13# Amended and Restated Employment Agreement between the Registrant and Jonathan Pachter, dated January 13, 2012 (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)
- 10.13# Form of Indemnification Agreement between the Registrant and each director (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed by the Registrant on August 8, 2017)
- 10.14 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 filed by the Registrant on April 18, 2014)
- 10.15# Employment Agreement, dated March 1, 2012, between the Registrant and Daniel Paterson (incorporated by reference to Exhibit 10.18 to the Annual Report on Form 10-K filed by the Registrant on March 26, 2013)
- 10.16† License Agreement, dated July 11, 2012, by and between the Registrant and Pfizer Inc. (incorporated by reference to Exhibit 10.2 to the Quarterly Report on Form 10-Q filed by the Registrant on August 13, 2012)
- 10.17# Letter Agreement, dated June 6, 2013, by and between the Registrant and Robert Forrester (incorporated by reference to Exhibit 10.2 to the Quarterly Report on Form 10-Q filed by the Registrant on August 13, 2013)
- 10.18† Letter Agreement, dated December 7, 2012, by and between the Registrant and Pfizer Inc. (incorporated by reference to Exhibit 10.31 to the Annual Report on Form 10-K filed by the Registrant on March 6, 2014)
- 10.19# Amended and Restated Employment Agreement, dated November 22, 2013, by and between the Registrant and Robert Forrester (incorporated by reference to Exhibit 10.32 to the Annual Report on Form 10-K filed by the Registrant on March 6, 2014)
- 10.20# Employment Agreement between the Registrant and Gregory Berk, dated April 15, 2016 (incorporated by reference to Exhibit 10.2 to the Quarterly Report on Form 10-Q filed by the Registrant on May 9, 2016)
- 10.21# Employment Agreement between the Registrant and Julie B. Feder, dated July 10, 2017 (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed by the Registrant on July 11, 2017)
- 10.22# Employment Agreement between the Registrant and NgocDiep T. Le, dated October 9, 2017 (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed by the Registrant on November 7, 2017)
- 10.23# Separation Agreement between the Registrant and Gregory Berk, effective January 19, 2017 (incorporated by reference to Exhibit 10.23 to the Annual Report on Form 10-K filed by the Registrant on March 23, 2017)
- 10.24# Consulting Agreement between the Registrant and Gregory Berk, effective January 20, 2017 (incorporated by reference to Exhibit 10.24 to the Annual Report on Form 10-K filed by the Registrant on March 23, 2017)

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10.25‡	<u>Amended and Restated License Agreement, dated November 1, 2016, by and between the Registrant and Infinity Pharmaceuticals, Inc. (incorporated by reference to Exhibit 10.25 to the Annual Report on Form 10-K filed by the Registrant on March 23, 2017)</u>
10.26	<u>Loan and Security Agreement, dated March 21, 2017, by and between the Registrant, the Lender (as defined therein) and Hercules Capital, Inc. (incorporated by reference to Exhibit 10.26 to the Annual Report on Form 10-K filed by the Registrant on March 23, 2017)</u>
10.27	<u>First Amendment to Loan and Security Agreement, dated January 4, 2018, by and between the Registrant, the Lender (as defined therein) and Hercules Capital, Inc. (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed by the Registrant on January 4, 2018)</u>
10.28*	<u>Second Amendment to Loan and Security Agreement, dated March 6, 2018, by and between the Registrant, the Lender (as defined therein) and Hercules Capital, Inc.</u>
10.29*#	<u>Employment Agreement between the Registrant and Joseph Lobacki, dated January 3, 2018</u>
21.1*	<u>Subsidiaries of the Registrant</u>
23.1*	<u>Consent of Ernst &amp; Young LLP</u>
31.1*	<u>Certification of the Chief Executive Officer pursuant to Exchange Act Rule 13a-14(a)</u>
31.2*	<u>Certification of the Chief Financial Officer pursuant to Exchange Act Rule 13a-14(a)</u>
32.1*	<u>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</u>
32.2*	<u>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</u>
99.1*	<u>Press Release issued by Verastem, Inc. on March 13, 2018 (furnished herewith)</u>
101.INS*	XBRL Instance Document
101.SCH*	XBRL Taxonomy Extension Schema Document
101.CAL*	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF*	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB*	XBRL Taxonomy Extension Label Linkbase Document
101.PRE*	XBRL Taxonomy Extension Presentation Linkbase Document

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\*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.

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## SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized on this 13th day of March 2018.

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 13, 2018
/s/ Julie B. Feder Julie B. Feder	Chief Financial Officer (Principal financial and accounting officer)	March 13, 2018
/s/ Timothy Barberich Timothy Barberich	Director	March 13, 2018
/s/ Michael Kauffman, M.D., Ph.D. Michael Kauffman, M.D., Ph.D.	Director	March 13, 2018
/s/ Alison Lawton Alison Lawton	Director	March 13, 2018
/s/ S. Louise Phanstiel S. Louise Phanstiel	Director	March 13, 2018
/s/ Eric Rowinsky, M.D. Eric Rowinsky, M.D.	Director	March 13, 2018
/s/ Brian Stuglik, R.Ph, Brian Stuglik, R.Ph.	Director	March 13, 2018
/s/ Bruce Wendel Bruce Wendel	Director	March 13, 2018



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Verastem, Inc.

CONSOLIDATED FINANCIAL STATEMENTS

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Verastem, Inc.

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of Verastem, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Verastem, Inc. (the Company) as of December 31, 2017 and 2016, the related consolidated statements of operations and comprehensive loss, stockholders' equity and cash flows for each of the three years in the period ended December 31, 2017, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2017 and 2016, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2017, in conformity with U.S. generally accepted accounting principles.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the Company's internal control over financial reporting as of December 31, 2017, based on criteria established in Internal Control-Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework), and our report dated March 13, 2018 expressed an unqualified opinion thereon.

The Company's Ability to Continue as a Going Concern

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 1 to the financial statements, the Company has an accumulated deficit, recurring losses, and expects continuing future losses, and has stated that substantial doubt exists about the Company's ability to continue as a going concern. Management's evaluation of the events and conditions and management's plans regarding these matters are also described in Note 1. The consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2011.

Boston, Massachusetts

March 13, 2018

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Verastem, Inc.

## CONSOLIDATED BALANCE SHEETS

(in thousands, except per share amounts)

	December 31,	
	2017	2016
Assets		
Current assets:		
Cash and cash equivalents	\$ 82,176	\$ 32,349
Short-term investments	4,496	48,548
Prepaid expenses and other current assets	1,115	398
Total current assets	87,787	81,295
Property and equipment, net	861	1,417
Restricted cash	162	162
Other assets	981	755
Total assets	\$ 89,791	\$ 83,629
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	\$ 9,186	\$ 4,095
Accrued expenses	7,942	6,896
Total current liabilities	17,128	10,991
Non-current liabilities:		
Long-term debt	14,828	—
Other non-current liabilities	151	341
Total liabilities	32,107	11,332
Stockholders' equity:		
Preferred stock, \$0.0001 par value; 5,000 shares authorized, no shares issued and outstanding at December 31, 2017 and 2016, respectively	—	—
Common stock, \$0.0001 par value; 100,000 shares authorized, 50,801 and 36,992 shares issued and outstanding at December 31, 2017 and 2016, respectively	5	4
Additional paid-in capital	360,823	307,587
Accumulated other comprehensive (loss) income	(2)	29
Accumulated deficit	(303,142)	(235,323)
Total stockholders' equity	57,684	72,297
Total liabilities and stockholders' equity	\$ 89,791	\$ 83,629
See accompanying notes to the consolidated financial statements.		

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Verastem, Inc.

## CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(in thousands, except per share amounts)

	Year Ended December 31,		
	2017	2016	2015
Operating expenses:			
Research and development	\$ 46,423	\$ 19,779	\$ 40,565
General and administrative	21,381	17,223	17,634
Total operating expenses	67,804	37,002	58,199
Loss from operations	(67,804)	(37,002)	(58,199)
Interest income	561	562	334
Interest expense	(559)	—	—
Net loss	\$ (67,802)	\$ (36,440)	\$ (57,865)
Net loss per share—basic and diluted	\$ (1.76)	\$ (0.99)	\$ (1.61)
Weighted-average number of common shares used in net loss per share—basic and diluted	38,422	36,988	35,932
Net loss	\$ (67,802)	\$ (36,440)	\$ (57,865)
Unrealized (loss) gain on available-for-sale securities	(31)	(14)	32
Comprehensive loss	\$ (67,833)	\$ (36,454)	\$ (57,833)
See accompanying notes to the consolidated financial statements.			

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Verastem, Inc.

## CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY

(in thousands, except share data)

	Common stock Shares	Amount	Additional paid-in capital	Accumulated other comprehensive (loss) income	Accumulated deficit	Total stockholders' equity
Balance at December 31, 2014	27,259,372	\$ 3	\$ 229,770	\$ 11	\$ (141,018)	\$ 88,766
Net loss	—	—	—	—	(57,865)	(57,865)
Unrealized gain on available-for-sale marketable securities	—	—	—	32	—	32
Issuance of common stock resulting from follow-on offering	8,337,500	1	50,941	—	—	50,942
Issuance of common stock resulting from at-the-market transactions, net of issuance costs of \$53	1,189,479	—	10,911	—	—	10,911
Vesting of restricted stock	7,995	—	2	—	—	2
Issuance of common stock resulting from exercise of stock options	33,658	—	13	—	—	13
Issuance of common stock resulting from vesting of restricted stock units and payment of tax withholdings	113,257	—	(417)	—	—	(417)
Stock-based compensation expense	—	—	10,085	—	—	10,085
Balance at December 31, 2015	36,941,261	\$ 4	\$ 301,305	\$ 43	\$ (198,883)	\$ 102,469
Net loss	—	—	—	—	(36,440)	(36,440)
Unrealized loss on available-for-sale marketable securities	—	—	—	(14)	—	(14)
Issuance of common stock resulting from exercise of stock options	1,605	—	—	—	—	—
Issuance of common stock resulting from vesting of restricted stock	49,552	—	(5)	—	—	(5)



units and payment of tax withholdings						
Stock-based compensation expense	—	—	6,287	—	—	6,287
Balance at December 31, 2016	36,992,418	\$ 4	\$ 307,587	\$ 29	\$ (235,323)	\$ 72,297
Net loss	—	—	—	—	(67,802)	(67,802)
Unrealized loss on available-for-sale marketable securities	—	—	—	(31)	—	(31)
Issuance of common stock resulting from follow-on offering, net of issuance costs of \$324	8,422,877	1	24,691	—	—	24,692
Issuance of common stock resulting from at-the-market transactions, net of issuance costs of \$112	5,036,879	—	23,053	—	—	23,053
Issuance of common stock resulting from exercise of stock options	348,734	—	442	—	—	442
Stock-based compensation expense	—	—	5,050	—	(17)	5,033
Balance at December 31, 2017	50,800,908	\$ 5	\$ 360,823	\$ (2)	\$ (303,142)	\$ 57,684
See accompanying notes to the consolidated financial statements.						

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Verastem, Inc.

## CONSOLIDATED STATEMENTS OF CASH FLOWS

(in thousands)

	Year ended December 31,		
	2017	2016	2015
Operating activities			
Net loss	\$ (67,802)	\$ (36,440)	\$ (57,865)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation	556	670	754
Stock-based compensation expense	5,033	6,287	10,085
Amortization of deferred financing costs, debt discounts and premiums and discounts on available-for-sale marketable securities	223	(140)	264
Loss on disposal of fixed assets	—	—	46
Changes in operating assets and liabilities:			
Prepaid expenses, other current assets and other assets	(943)	(568)	276
Accounts payable	5,046	153	863
Accrued expenses and other liabilities	577	623	418
Liability classified stock-based compensation awards	—	(69)	(400)
Net cash used in operating activities	(57,310)	(29,484)	(45,559)
Investing activities			
Purchases of property and equipment	—	(39)	(211)
Purchases of investments	(7,957)	(82,101)	(199,851)
Maturities of investments	51,910	119,067	173,005
Decrease in restricted cash	—	41	—
Net cash provided by (used in) investing activities	43,953	36,968	(27,057)
Financing activities			
Proceeds from long-term debt, net	14,811	—	—
Deferred debt financing costs	(138)	—	—
Proceeds from the exercise of stock options	442	—	13
Proceeds from the issuance of common stock, net	48,069	—	63,989
Cash used to settle restricted stock liability	—	(5)	(417)
Net cash provided by (used in) financing activities	63,184	(5)	63,585
Increase (decrease) in cash and cash equivalents	49,827	7,479	(9,031)
Cash and cash equivalents at beginning of period	32,349	24,870	33,901
Cash and cash equivalents at end of period	\$ 82,176	\$ 32,349	\$ 24,870
Supplemental disclosures			
Cash paid for interest	\$ 295	\$ —	\$ —
Supplemental disclosure non-cash financing activities			
Common stock issuance costs included in accounts payable and accrued expenses	\$ 324	\$ —	\$ —

See accompanying notes to the consolidated financial statements.

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### 1. Nature of business

Verastem, Inc. (the Company) is a biopharmaceutical company focused on developing and commercializing drugs to improve outcomes for patients with cancer. 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 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, 2017, the Company had cash, cash equivalents and investments of \$86.7 million and accumulated deficit of \$303.1 million. The Company has historical losses from operations and anticipates that it will continue to incur losses for the foreseeable future as it continues the research and development and clinical trials of, and seeks marketing approval for, its lead product candidates. Without additional funding, the Company believes that it will not have sufficient funds to meet its obligations within the next twelve months from the date of issuance of these consolidated financial statements. These factors raise substantial doubt about the Company's ability to continue as a going concern.

The Company plans to continue to fund its operations through proceeds from sales of its common stock under its at-the-market equity offering program, public or private equity offerings, its loan and security agreement with Hercules Capital, Inc. (Hercules), or other strategic transactions. However, adequate additional financing may not be available to the Company on acceptable terms, or at all. If the Company is unable to raise capital when needed or on attractive terms, it may be forced to delay, reduce or eliminate its research and development programs or any future commercialization efforts.

### 2. Significant accounting policies

#### Basis of presentation

The accompanying financial statements of the Company have been prepared in accordance with U.S. generally accepted accounting principles (GAAP) under the assumption that the Company will continue as a going concern for the next twelve months. Accordingly, they do not include any adjustments that might result from the uncertainty related to the Company's ability to continue as a going concern.

The consolidated financial statements include the accounts of Verastem Securities Company, a wholly-owned subsidiary of the Company. 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.

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## Segment and geographic information

Operating segments are defined as components of an enterprise about which separate discrete information is available and regularly reviewed 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 for the treatment of cancer. All material long-lived assets of the Company reside in the United States.

## 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 a U.S. Government money market fund, overnight repurchase agreements collateralized by government agency securities or U.S. Treasury securities, corporate bonds and commercial paper of publicly traded companies. Cash equivalents are reported at fair value.

## Fair value of financial instruments

The Company determines the fair value of its financial instruments based upon the fair value hierarchy, which 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 that the Company can access at the measurement date.
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.

## Items Measured at Fair Value on a Recurring Basis

The following table presents information about the Company's financial instruments that are measured at fair value on a recurring basis (in thousands):

Description	December 31, 2017			
	Total	Level 1	Level 2	Level 3
Financial assets				
Cash equivalents	\$ 80,894	\$ 75,478	\$ 5,416	\$ —
Short-term investments	4,496	—	4,496	—
Total financial assets	\$ 85,390	\$ 75,478	\$ 9,912	\$ —

Description	December 31, 2016			
	Total	Level 1	Level 2	Level 3
Financial assets				
Cash equivalents	\$ 30,540	\$ 20,540	\$ 10,000	\$ —
Short-term investments	48,548	—	48,548	—
Total financial assets	\$ 79,088	\$ 20,540	\$ 58,548	\$ —

These investments and cash equivalents have been initially valued at the transaction price and subsequently valued, at the end of each reporting period, utilizing third party pricing services or other market observable data. The

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pricing services utilize industry standard valuation models, including both income and market based approaches and observable market inputs to determine value. These observable market inputs include reportable trades, benchmark yields, credit spreads, broker/dealer quotes, bids, offers, current spot rates and other industry and economic events. The Company validates the prices provided by third party pricing services by reviewing their pricing methods and matrices, obtaining market values from other pricing sources, analyzing pricing data in certain instances and confirming that the relevant markets are active. After completing its validation procedures, the Company did not adjust or override any fair value measurements provided by the pricing services as of December 31, 2017 and 2016.

## Fair Value of Financial Instruments

The fair value of the Company's long-term debt is determined using current applicable rates for similar instruments as of the balance sheet dates and an assessment of the credit rating of the Company. The carrying value of the Company's debt at December 31, 2017 approximates fair value because the Company's interest rate yield is near current market rates for comparable debt instruments. The fair value of the Company's long-term debt was determined using Level 3 inputs.

## Investments

Investments and cash equivalents consist of investments in a U.S. Government money market fund, overnight repurchase agreements collateralized by government agency securities or U.S. Treasury securities, corporate bonds and commercial paper of publicly traded companies that are classified as available for sale pursuant to Accounting Standards Codification (ASC) Topic 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 consolidated statements of operations and comprehensive loss.

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. Realized gains and losses are determined using the specific identification method and are included in interest income in the consolidated statements of operations and comprehensive loss.

There were no realized gains or losses on investments for the years ended December 31, 2017, 2016 or 2015. There were no investments that had been in an unrealized loss position for more than 12 months as of December 31, 2017 or December 31, 2016. There were 5 debt securities in an unrealized loss position for less than 12 months at



December 31, 2017 and there were 14 debt securities that had been in an unrealized loss position for less than 12 months at December 31, 2016. The aggregate unrealized loss on these securities as of December 31, 2017 and December 31, 2016 was approximately \$2,000 and \$24,000, respectively, and the fair value was \$9.9 million and \$23.6 million, respectively. The Company considered the decline in the market value for these securities to be primarily attributable to current economic conditions. As it was not more likely than not that the Company would be required to sell these securities before the recovery of their amortized cost basis, which may be at maturity, the Company did not consider these investments to be other-than-temporarily impaired as of December 31, 2017.

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Cash, cash equivalents and investments consist of the following (in thousands):

	December 31, 2017			Fair Value
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	
Cash and cash equivalents:				
Cash and money market accounts	\$ 76,760	\$ —	\$ —	\$ 76,760
Corporate bonds and commercial paper	\$ 5,418	\$ —	\$ (2)	\$ 5,416
Total cash and cash equivalents	\$ 82,178	\$ —	\$ (2)	\$ 82,176
Investments:				
Corporate bonds and commercial paper (due within 1 year)	\$ 4,496	\$ —	\$ —	\$ 4,496
Total investments	\$ 4,496	\$ —	\$ —	\$ 4,496
Total cash, cash equivalents, and investments	\$ 86,674	\$ —	\$ (2)	\$ 86,672

	December 31, 2016			Fair Value
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	
Cash and cash equivalents:				
Cash and money market accounts	\$ 22,349	\$ —	\$ —	\$ 22,349
Overnight repurchase agreements	10,000	—	—	10,000
Total cash and cash equivalents	\$ 32,349	\$ —	\$ —	\$ 32,349
Investments:				
Corporate bonds and commercial paper (due within 1 year)	\$ 48,519	\$ 53	\$ (24)	\$ 48,548
Total investments	\$ 48,519	\$ 53	\$ (24)	\$ 48,548
Total cash, cash equivalents, and investments	\$ 80,868	\$ 53	\$ (24)	\$ 80,897

#### 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. The Company mitigates this risk by maintaining its cash and cash equivalents and investments with high quality, accredited financial institutions. The management of the Company's investments is not discretionary on the part of these financial institutions. As of December 31, 2017, the Company's cash, cash equivalents and investments were deposited at two financial institutions and it has no significant off balance sheet concentrations of credit risk, such as foreign currency exchange contracts, option contracts or other hedging arrangements.

#### Property and equipment

Property and equipment consists of laboratory equipment, office furniture, 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 and amortization 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

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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 material impairment losses have been recorded through December 31, 2017.

### Other assets

Other assets primarily consists of prepayments made to contract research organizations (CROs). As of December 31, 2017 and 2016, other assets was primarily comprised of approximately \$755,000 of prepaid CRO expenses that the Company assumed and paid to Infinity Pharmaceuticals, Inc. (Infinity) pursuant to the license agreement between the Company and Infinity.

### Research and development costs

The Company expenses research and development costs to operations as incurred. 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 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.

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 services have been performed or when the goods have been received rather than when the payment is made.

### Stock based compensation

The Company expenses the fair value of employee stock-based awards on a straight-line basis over the requisite service period, which typically 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 to reflect actual forfeitures as they occur. 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.

The grant date fair value of employee stock options is estimated using the Black-Scholes option pricing model that takes into account the fair value of its common stock, the exercise price, the expected life of the option, the expected volatility of its common stock, expected dividends on its common stock, and the risk-free interest rate over the expected life of the option. The Company uses the simplified method described in 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 five companies, including the Company and a representative group of four public biotechnology and life sciences companies with similar characteristics to the Company, including similar stage of product development and therapeutic focus. The risk free interest rate is based on a treasury instrument whose term is consistent with the expected term of the stock options. Historically, the Company has recognized stock-based compensation net of estimated forfeitures over the vesting period of the respective grant. Effective January 1, 2017, the Company adopted Accounting Standard Updated (ASU) 2016-09, Compensation – Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting, which simplified the accounting for share-based compensation arrangements, including the accounting

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for forfeitures. Upon adoption, the Company elected to begin accounting for forfeitures as they occur, rather than estimating a forfeiture rate, and recorded an immaterial cumulative-effect adjustment to opening accumulated deficit.

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 to non-employees 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 allowed for greater than the minimum statutory tax withholdings were classified as liabilities. These awards were revalued at each reporting date and were expensed on a straight line basis over the vesting period. Upon settlement, the awards were revalued and the amounts were reclassified to additional paid in capital. Shares were withheld to cover the tax withholding and amounts paid to settle the tax liability were 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.

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

	Year ended December 31,		
	2017	2016	2015
Outstanding stock options	8,719,978	5,848,470	5,390,130
Outstanding warrants	—	142,857	142,857
Unvested restricted stock units	—	—	53,751
Total potentially dilutive securities	8,719,978	5,991,327	5,586,738

Recently Issued Accounting Standards Updates

In May 2017, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) 2017-09, Compensation – Stock Compensation (Topic 718): Scope of Modification Accounting. ASU 2017-09 provides guidance about which changes to the terms or conditions of a share-based award require an entity to apply modification accounting under Topic 718. Specifically, an entity would not apply modification accounting if the fair value, vesting conditions and classification of the awards are the same immediately before and after a modification. ASU 2017-09 is effective for annual and interim periods beginning after December 15, 2017, with early adoption permitted. The Company has not elected to early adopt this standard and does not expect the adoption to have a material impact on its consolidated financial statements and related disclosures.

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In November 2016, the FASB issued ASU 2016-18, Statement of Cash Flows (Topic 230): Restricted Cash. ASU 2016-18 requires that a statement of cash flows explain the change during the period in the total of cash, cash equivalents and amounts generally described as restricted cash or restricted cash equivalents. Amounts generally described as restricted cash and restricted cash equivalents should be included with cash and cash equivalents when reconciling the beginning-of-period and end-of-period total amounts shown on the statement of cash flows. ASU 2016-18 is effective for annual and interim periods beginning after December 15, 2017, with early adoption permitted. The Company has not elected to early adopt this standard and does not expect the adoption to have a material impact on its consolidated financial statements and related disclosures.

In August 2016, the FASB issued ASU 2016-15, Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments. ASU 2016-15 adds or clarifies guidance on the classification of certain cash receipts and payments in the statement of cash flows. The standard is effective for annual and interim periods beginning after December 15, 2017, with early adoption permitted. The Company has not elected to early adopt this standard and does not expect the adoption to have a material impact on its consolidated financial statements and related disclosures.

In February 2016, the FASB issued ASU 2016-02, Leases (Topic 842), which supersedes the guidance under FASB Accounting Standards Codification (ASC) Topic 840, Leases, resulting in the creation of FASB ASC Topic 842, Leases. ASU 2016-02 requires lessees to recognize in the statement of financial position a liability to make lease payments and a right-of-use asset representing its right to use the underlying asset for the lease term for both finance and operating leases. The guidance also eliminates the current real estate-specific provisions for all entities. ASU 2016-02 is effective for fiscal years, and interim periods within those years, beginning after December 15, 2018, with early adoption permitted. The Company has not elected to early adopt this standard and is currently evaluating the impact the adoption of the standard will have on its consolidated financial statements and related disclosures.

Recently Adopted Accounting Standards Updates

In January 2017, the FASB issued ASU 2017-03, Accounting Changes and Error Corrections (Topic 250) and Investments – Equity Method and Joint Ventures (Topic 323): Amendments to SEC Paragraphs Pursuant to Staff Announcements at the September 22, 2016 and November 17, 2016 EITF Meetings. ASU 2017-03 clarifies the SEC staff's expectations about the extent of disclosures that a registrant is expected to provide regarding the impact that the adoption of ASUs 2014-09 (Revenue from Contracts with Customers), 2016-02 (Leases) and 2016-13 (Measurement of Credit Losses on Financial Instruments) will have on its financial statements. It also conforms SEC guidance on accounting for tax benefits resulting from investments in affordable housing projects to the guidance in ASU 2014-01, Investments -Equity Method and Joint Ventures (Topic 323). The guidance under this ASU was effective upon issuance and did not have a material impact on the Company's disclosures.

In October 2016, the FASB issued ASU 2016-17, Consolidation (Topic 810): Interests Held through Related Parties That Are under Common Control. ASU 2016-17 updates ASU 2015-02. Under the amendments, a single decision maker is not required to consider indirect interests held through related parties that are under common control with the single decision maker to be the equivalent of direct interests in their entirety. Instead, a single decision maker is required to include those interests on a proportionate basis consistent with indirect interests held through other related parties. ASU 2016-17 is effective for annual and interim periods beginning after December 15, 2016. The Company adopted this standard effective January 1, 2017. The adoption of this ASU did not have an effect on the Company's



financial statements or disclosures.

In March 2016, the FASB issued ASU 2016-09, Compensation – Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting. ASU 2016-09 simplifies the accounting for share-based compensation arrangements, including the accounting for forfeitures, income tax consequences, classification of awards as either equity or liabilities and classification on the statement of cash flows. The standard was effective for annual and interim periods beginning after December 15, 2016, with early adoption permitted. The Company adopted ASU 2016-09 effective January 1, 2017. Upon adoption, the Company elected to begin accounting for forfeitures as they occur, rather than estimating a forfeiture rate, and recorded an immaterial cumulative-effect adjustment to opening accumulated deficit. Also upon adoption, the Company recognized all previously

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unrecognized tax benefits, which would have resulted in the recognition of an immaterial cumulative-effect adjustment to opening accumulated deficit; however, these unrecognized tax benefits were recorded as a deferred tax asset, which was fully offset by a valuation allowance. Therefore, the recognition of these benefits had no net cumulative-effect on opening accumulated deficit upon adoption.

## 3. Property and equipment, net

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

	December 31, 2017	December 31, 2016
Leasehold improvements	\$ 2,104	\$ 2,104
Laboratory equipment	908	908
Furniture and fixtures	325	325
Computer equipment	279	279
	3,616	3,616
Less: accumulated depreciation	(2,755)	(2,199)
Total property, plant and equipment, net	\$ 861	\$ 1,417

Approximate total depreciation and amortization expenses amounted to \$556,000, \$670,000, and \$754,000 for the years ended December 31, 2017, 2016, and 2015, respectively.

## 4. Accrued expenses

Accrued expenses consist of the following (in thousands):

	December 31, 2017	December 31, 2016
Contract research organization costs	\$ 3,774	\$ 3,258
Compensation and related benefits	2,622	2,505
Professional fees	617	403
Consulting fees	579	527
Deferred rent	190	175
Other	160	28
Total accrued expenses	\$ 7,942	\$ 6,896

## 5. Long-term debt

On March 21, 2017 (Closing Date), Verastem, Inc. (the Borrower) entered into a term loan facility of up to \$25.0 million with Hercules, a Maryland corporation, the proceeds of which will be used for its ongoing research and development programs and for general corporate purposes. The term loan facility is governed by a loan and security agreement, dated March 21, 2017 (the Original Loan Agreement), which originally provided for up to four separate advances, of which the first tranche of \$2.5 million was drawn on the Closing Date. The second and third tranches of \$2.5 million and \$5.0 million, respectively were drawn on October 12, 2017 after announcing favorable data from the Company's Phase III clinical study evaluating the safety and efficacy of duvelisib in patients with relapsed/refractory chronic lymphocytic leukemia or small lymphocytic lymphoma. A total of \$6.0 million of the proceeds received from the second and third tranches were used to make a milestone payment pursuant to the Company's license agreement with Infinity. The fourth tranche of \$15.0 million could be drawn, at the Borrower's option and at the sole discretion of Hercules, on or prior to June 30, 2018. On December 20, 2017, the Borrower drew an additional advance on the fourth tranche of \$5.0 million.

On January 4, 2018, the Borrower entered into the First Amendment to the Original Loan Agreement (the First Amendment) and on March 6, 2018, the Borrower entered into the Second Amendment to the Original Loan Agreement (the Second Amendment, and the Original Loan Agreement as amended by the First Amendment and the Second Amendment, the Amended Loan Agreement). The First Amendment increased the borrowing limit under the Original Loan Agreement from up to \$25.0 million to up to \$50.0 million (the Term Loan). As \$15.0 million in

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term loans had already been drawn prior to entering into the First Amendment, there is \$35.0 million of borrowing capacity remaining under the Amended Loan Agreement. The remaining \$35.0 million of borrowing capacity may be drawn in minimum increments of \$5.0 million in multiple tranches comprised of (i) term loans (each a Term E Loan Advance) in an aggregate principal amount of up to \$10.0 million and (ii) subject to Hercules' sole discretion, term loans (each a Term F Loan Advance) in an aggregate principal amount of up to \$25.0 million. The Amended Loan Agreement permits the Borrower to draw Term E Loan Advances subject to (i) the U.S. Food and Drug Administration accepting on or prior to September 30, 2018 our New Drug Application for duvelisib and (ii) delivery of the Company's financial and business projections to Hercules in form and substance reasonably acceptable to Hercules. In addition, the Amended Loan Agreement allows the Borrower to draw Term F Loan Advances subject to the prior drawing of all other tranches and Hercules' sole discretion.

The Term Loan will mature on December 1, 2020 (Loan Maturity Date). Each advance accrues interest at a floating per annum rate equal to the greater of either (a) 10.5% or (b) the lesser of (i) 12.75% and (ii) the sum of (x) 10.5% plus (y) (A) the prime rate minus (B) 4.5%. The Term Loan provided for interest-only payments until November 1, 2018, which was extended to May 1, 2019 pursuant to the Amended Loan Agreement upon the Borrower's receipt of a minimum of \$20.0 million cash proceeds from a sale of equity securities in December 2017. Thereafter, amortization payments will be payable monthly in twenty installments of principal and interest (subject to recalculation upon a change in prime rates). Any advance may be prepaid in whole or in part upon seven business days' prior written notice to Hercules, subject to a prepayment charge of 3.0%, if such advance is prepaid in any of the first twelve (12) months following the Closing Date, 2.0%, if such advance is prepaid after twelve (12) months following the Closing Date but on or prior to twenty-four (24) months following the Closing Date, and 1.0% thereafter. In addition, a final payment equal to 4.5% of the greater of (a) \$5.0 million and (b) the total principal amount of the Term Loan extended by Hercules which is due on the Loan Maturity Date, or such earlier date specified in the Amended Loan Agreement. Amounts outstanding during an event of default shall be payable on demand and shall accrue interest at an additional rate of 5.0% per annum of the past due amount outstanding.

The Term Loan is secured by a lien on substantially all of the assets of the Borrower, other than intellectual property and contains customary covenants and representations, including a liquidity covenant, financial reporting covenant and limitations on dividends, indebtedness, collateral, investments, distributions, transfers, mergers or acquisitions, taxes, corporate changes, deposit accounts, and subsidiaries.

The events of default under the Amended Loan Agreement include, without limitation, and subject to customary grace periods, (1) the Borrower's failure to make any payments of principal or interest under the Amended Loan Agreement, promissory notes or other loan documents, (2) the Borrower's breach or default in the performance of any covenant under the Amended Loan Agreement, (3) the Borrower making a false or misleading representation or warranty in any material respect, (4) the Borrower's insolvency or bankruptcy, (5) certain attachments or judgments on the Borrower's assets, or (6) the occurrence of any material default under certain agreements or obligations of the Borrower involving indebtedness, or (7) the occurrence of a material adverse effect. If an event of default occurs, Hercules is entitled to take enforcement action, including acceleration of amounts due under the Amended Loan Agreement.

The Company assessed all terms and features of the Original Loan Agreement in order to identify any potential embedded features that would require bifurcation or any beneficial conversion features. As part of this analysis, the Company assessed the economic characteristics and risks of the Original Loan Agreement, including put and call

features. The Company determined that all features of the Original Loan Agreement were clearly and closely associated with a debt host and did not require bifurcation as a derivative liability, or the fair value of the feature was immaterial to the Company's financial statements. The Company reassesses the features on a quarterly basis to determine if they require separate accounting.

The future principal payments under the Amended Loan Agreement are as follows as of December 31, 2017 (in thousands):

2018	\$ —
2019	3,609
2020	11,391

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Total principal payments \$ 15,000

## 6. Common stock

As of December 31, 2017 and 2016, 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, 2017	December 31, 2016
Shares reserved under equity compensation plans	8,264	7,189
Shares reserved for inducement grants	3,616	1,255
Shares reserved for outstanding warrants	—	143
Total shares reserved	11,880	8,587

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.

## At-the-market equity offering programs

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 \$35.0 million of its common stock at then-current market prices from time to time through Cantor Fitzgerald & Co. (Cantor), as sales agent (the 2013 ATM Program). In November 2014, the Company commenced sales under the 2013 ATM Program. During the year ended December 31, 2015, the Company sold 1,189,479 shares of common stock under the 2013 ATM Program with net proceeds (after deducting commissions and other offering expenses) of \$12.9 million. No proceeds were received and no additional sales of the Company's common stock were made under the 2013 ATM Program and no proceeds were received during the years ended December 31, 2017 and 2016.

On March 30, 2017, the Company terminated the 2013 ATM Program and established a new at-the-market equity offering program pursuant to which it was able to offer and sell up to \$35.0 million of its common stock at then-current market prices from time to time through Cantor, as sales agent (the 2017 ATM Program). On August 28, 2017, the Company amended its sales agreement with Cantor to increase the maximum aggregate offering price of shares of common stock that can be sold under the 2017 ATM Program to \$75.0 million. Through December 31, 2017, the Company sold 5,036,879 shares under the 2017 ATM Program for net proceeds of approximately \$23.1 million (after deducting commissions and other offering expenses).

As of March 13, 2018, the Company sold an additional 97,078 shares of common stock under the at-the-market equity offering program with net proceeds of approximately \$342,000 (after deducting commissions and other offering expenses).

## Equity offering

On December 14, 2017, the Company entered into an Underwriting Agreement with BTIG, LLC relating to the underwritten offering of 8,422,877 shares of its common stock at a price of \$2.97 per share, for aggregate proceeds,

net of underwriting discounts and offering costs, of approximately \$24.7 million.

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## 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,		
	2017	2016	2015
Research and development	\$ 1,381	\$ 1,073	\$ 2,411
General and administrative	3,652	5,145	7,273
Total stock-based compensation expense	\$ 5,033	\$ 6,218	\$ 9,684

All of the \$5.0 million of stock-based compensation expense recorded during the year ended December 31, 2017 was recorded to additional paid-in capital. Of the \$6.2 million of stock-based compensation expense recorded during the year ended December 31, 2016, \$6.3 million was recorded to additional paid-in capital and approximately \$69,000 was recorded as a decrease in liability classified awards. Of the \$9.7 million of stock-based compensation expense recorded during the year ended December 31, 2015, \$10.1 million was recorded to additional paid-in capital and approximately \$400,000 was recorded as a decrease 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), as well as the inducement award program. 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 for a period of ten years from the date of grant.

**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 initially allowed 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 is added on the first day of each year beginning in 2013 and each subsequent anniversary until the expiration of the 2012 Plan, and is equal to the lower 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, 2017 and 2016, the number of shares available for issuance under the 2012 Plan increased by 1,285,714 under this provision. Subsequently, on January 1, 2018, the number of shares available for issuance under the 2012 Plan increased by 1,285,714 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, 2017, under the 2012 Plan, the Company has granted stock options for 10,684,203 shares of common stock, of which 3,005,939 have been forfeited and 137,127 have been exercised, and restricted stock units for 909,918 shares of common stock, of which 150,101 have been forfeited. The exercise price of each option has been equal to the closing price of a share of our common



stock on the grant date.

#### Inducement Award Program

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

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Company. In December 2016, the Board of Directors authorized and reserved 580,000 additional shares of common stock under this program. In December 2017, the Board of Directors authorized and reserved 2,500,000 additional shares of common stock under this program. The program is governed by the terms of the 2012 Plan but does not fall under the 2012 Plan. As of December 31, 2017, the Company had granted options for 1,324,000 shares of common stock under the program, of which 75,000 have been forfeited and 138,750 have been exercised. As of December 31, 2017, 2,506,000 remain available for future issuance.

## Stock Options

A summary of the Company's stock option activity and related information for the year ended December 31, 2017 is as follows:

	Shares	Weighted-average exercise price per share	Weighted-average remaining contractual term (years)	Aggregate intrinsic value (in thousands)
Outstanding at December 31, 2016	5,848,470	\$ 6.35	8.0	\$ 62
Granted	3,553,430	\$ 2.60		
Exercised	(348,734)	\$ 1.27		
Forfeited/cancelled	(333,188)	\$ 2.04		
Outstanding at December 31, 2017	8,719,978	\$ 5.19	7.9	\$ 6,150
Vested at December 31, 2017	4,795,186	\$ 7.04	6.9	\$ 2,562
Vested and expected to vest at December 31, 2017(1)	8,649,978	\$ 5.20	7.9	\$ 6,150

(1) This represents the number of vested options as of December 31, 2017, plus the number of unvested options expected to vest as of December 31, 2017.

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

	Year ended December 31,		
	2017	2016	2015
Risk-free interest rate	2.02 %	1.48 %	1.75 %
Volatility	78 %	75 %	73 %
Dividend yield	—	—	—
Expected term (years)	5.8	5.9	6.0

The Company recorded stock based compensation expense associated with employee stock options of \$4.5 million, \$6.1 million, and \$7.9 million for the years ended December 31, 2017, 2016 and 2015, respectively. The weighted average grant date fair value of options granted in the years ended December 31, 2017, 2016 and 2015 was \$1.83, \$0.99, and \$3.80 per share, respectively. The fair value of options that vested during the years ended December 31, 2017, 2016 and 2015 was \$4.8 million, \$6.9 million, and \$9.5 million, respectively. The aggregate intrinsic value

of options exercised (i.e., the difference between the market price at exercise and the price paid by employees to exercise the option) during the years ended December 31, 2017 and 2016 was \$1.1 million and \$0, respectively.

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In June 2016, the Company granted stock options to purchase a total of 500,000 shares of common stock to certain employees that vest only upon the achievement of specified performance conditions. The Company determined that 50% of performance conditions had been achieved during the year ended December 31, 2016. As a result, 250,000 shares vested in October 2016 and the Company recognized stock-based compensation expense related to these awards of approximately \$222,000 for the year ended December 31, 2016. In September 2017, the Company determined that the remaining performance conditions had been achieved and as a result the remaining 250,000 shares vested and the Company recognized stock-based compensation expense of approximately \$379,000 during the year ended December 31, 2017. The increase in stock-based compensation expense recognized for the awards which vested during the year ended December 31, 2017, as compared to the awards which vested during the year ended December 31, 2016, is a result of the revaluation of an award held by a non-employee to fair value on the vesting date.

At December 31, 2017, there was \$6.8 million of total unrecognized compensation cost related to unvested stock options and the Company expects to recognize this cost over a remaining weighted-average period of 2.9 years.

### Restricted Stock Units

No restricted stock units were granted during the years ended December 31, 2017, 2016 and 2015. The total fair value of restricted stock units vested during the years ended December 31, 2017, 2016 and 2015 was approximately \$0, \$65,000 and \$1.6 million, respectively. As of December 31, 2016, all RSUs granted under the 2012 plan had vested.

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 were considered to be liability instruments until vested. As a result of this modification, the Company recorded a liability for the fair value of the awards as of each reporting date with the change in fair value recorded through the consolidated statements of operations and comprehensive loss. The Company recorded stock based compensation expense equal to the greater of the original grant date fair value of the awards or the settlement date fair value. All RSUs were fully vested as of February 1, 2016. During the year ended December 31, 2017, 2016 and 2015, the Company deposited approximately \$0, \$5,000, and \$417,000, respectively, with tax authorities to settle the tax liability for awards that settled during the respective periods. There was no liability related to these awards as of December 31, 2017 and 2016.

### 8. Income Taxes

As of December 31, 2017, the Company had federal and state net operating loss carryforwards of approximately \$237.0 million and \$235.9 million, respectively, which are available to reduce future taxable income. The Company also had federal and state tax credits of \$14.1 million and \$1.7 million, respectively, which may be used to offset future tax liabilities. The net operating loss (NOL) and tax credit carryforwards will expire at various dates through 2037. NOL 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.



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

	December 31,			
	2017		2016	
Income tax benefit using U.S. federal statutory rate	34.00	%	34.00	%
State tax benefit, net of federal benefit	5.00	%	3.43	%
Research and development tax credits	7.04	%	4.42	%
Permanent items	(1.24)	%	(3.88)	%
Effect of U.S. Tax Cuts and Jobs Act	(45.19)	%	—	%
Change in the valuation allowance	0.19	%	(36.71)	%
Other	0.20	%	(1.26)	%
	—	%	—	%

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

	December 31,	
	2017	2016
Deferred tax assets:		
Net operating loss carryforwards	\$ 64,677	\$ 72,285
Capitalized research and development	2,780	1,836
Research and development credits	15,406	8,298
Stock-based compensation	2,560	3,083
Other	379	429
Gross deferred tax assets	85,802	85,931
Valuation allowance	(85,802)	(85,931)
Net deferred tax asset	\$ —	\$ —

The Company has recorded a valuation allowance against its deferred tax assets at December 31, 2017 and 2016 because the Company's management believes that it is more likely than not that these assets will not be fully realized. The decrease in the valuation allowance of approximately \$129,000 in the year ended December 31, 2017 primarily relates to the reduction in the federal rate for corporations under the Tax Cuts and Jobs Act of 2017.

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, 2017, the Company had no unrecognized tax benefits or related interest and penalties accrued. The Company has not 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.

On December 22, 2017, the Tax Cuts and Jobs Act (TCJA) was enacted. This law substantially amended the Internal Revenue Code and among other things, effective January 1, 2018, permanently reduced the U.S. corporate income tax rate from 35% to 21% and repealed the performance exception permitting certain executive officer compensation greater than \$1.0 million to be deducted. On December 22, 2017, the SEC staff issued Staff Accounting Bulletin No. 118, Income Tax Accounting Implications of the Tax Cuts and Jobs Act (SAB 118), which allows the recording of provisional amounts during a measurement period not to extend beyond one year of the enactment date. In accordance with SAB 118, the Company has determined that its deferred tax asset value and

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associated valuation allowance reduction of \$30.6 million is a provisional amount and a reasonable estimate at December 31, 2017. The final impact may differ from this provisional amount due to, among other things, changes in interpretations and assumptions the Company has made thus far and the issuance of additional regulatory or other guidance. The Company expects to determine the final impact within the measurement period.

## 9. Commitments and contingencies

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 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 the lease incentive obligation are included in accrued expenses (current portion) and other liabilities (noncurrent portion) in the consolidated balance sheets. 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 was reduced to approximately \$162,000 on April 15, 2016. The amount is included in long term restricted cash on the consolidated balance sheets as of December 31, 2017 and 2016.

The minimum aggregate future lease commitments as of December 31, 2017 are as follows (in thousands):

2018	\$ 542
2019	415
2020	—
2021	—
2022	—
Thereafter	—
Total	\$ 957

The Company recorded rent expense of approximately \$352,000, \$352,000 and \$352,000 for the years ended December 31, 2017, 2016 and 2015, 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 November 2016, the Company entered into an amended and restated license agreement with Infinity, under which it acquired an exclusive worldwide license for the research, development, commercialization, and manufacture of products in oncology indications containing duvelisib. In connection with the license agreement, the Company assumed operational and financial responsibility for certain activities that were part of Infinity's duvelisib program, including the DUO study for patients with relapsed/refractory CLL, and Infinity assumed financial responsibility for the shutdown of certain other clinical studies up to a maximum of \$4.5 million. The Company is obligated to use diligent efforts to develop and commercialize a product in an oncology indication containing duvelisib. During the



term of the license agreement, Infinity has agreed not to research, develop, manufacture or commercialize duvelisib in any other indication in humans or animals.

Pursuant to the terms of the license agreement, the Company is required to make the following payments to

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Infinity in cash or, at our election, in whole or in part, in shares of our common stock: (i) \$6.0 million upon the completion of the DUO study if the results of the DUO study meet certain pre-specified criteria, which milestone was paid in cash by the Company to Infinity in October 2017 and recorded as research and development expense in the consolidated statement of operations, and (ii) \$22.0 million upon the approval of a New Drug Application in the United States or an application for marketing authorization with a regulatory authority outside of the United States for a product in an oncology indication containing duvelisib. For any portion of any of the foregoing payments that it elects to issue in shares of our common stock in lieu of cash, the number of shares of common stock to be issued will be determined by multiplying (1) 1.025 by (2) the number of shares of common stock equal to (a) the amount of the payment to be paid in shares of common stock divided by (b) the average closing price of a share of common stock as quoted on Nasdaq for a twenty day period following the public announcement of the applicable milestone event. The shares of common stock will be issued as unregistered securities, and the Company will have an obligation to promptly file a registration statement with the SEC to register such shares for resale. Any issuance of shares will be subject to the satisfaction of closing conditions, including that all material authorizations, consents, approvals and the like necessary for such issuance shall have been obtained.

The Company is also obligated to pay Infinity royalties on worldwide net sales of any products in an oncology indication containing duvelisib ranging from the mid-single digits to the high single-digits. The royalties will expire on a product-by-product and country-by-country basis until the latest to occur of (i) the last-to-expire patent right covering the applicable product in the applicable country, (ii) the last-to-expire patent right covering the manufacture of the applicable product in the country of manufacture of such product, (iii) the expiration of non-patent regulatory exclusivity in such country and (iv) ten years following the first commercial sale of a product in a country, provided that if royalties on net sales for a product in the United States are payable solely on the basis of non-patent regulatory exclusivity, the applicable royalty on net sales for such product in the United States will be reduced by 50%. The royalties are also subject to reduction by 50% of certain third-party royalty payments or patent litigation damages or settlements which might be required to be paid by us if litigation were to arise, with any such reductions capped at 50% of the amounts otherwise payable during the applicable royalty payment period.

In addition to the foregoing, the Company is obligated to pay Infinity an additional royalty of 4% on worldwide net sales of any products in an oncology indication containing duvelisib to cover the reimbursement of research and development costs owed by Infinity to Mundipharma International Corporation Limited (MICL) and Purdue Pharmaceutical Products L.P. (Purdue). Once Infinity has fully reimbursed MICL and Purdue, the royalty obligations will be reduced to 1% of net sales in the United States. These trailing MICL royalties are payable until the later to occur of the last-to-expire of specified patent rights and the expiration of non-patent regulatory exclusivities in a country. Each of the above royalty rates is reduced by 50% on a product-by-product and country-by-country basis if the applicable royalty is payable solely on the basis of non-patent regulatory exclusivity. In addition, the trailing MICL royalties are subject to reduction by 50% of certain third-party royalty payments or patent litigation damages or settlements which might be required to be paid by the us if litigation were to arise, with any such reductions capped at 50% of the amounts otherwise payable during the applicable royalty payment period.

The Company evaluated the license agreement with Infinity under ASC Topic 805, Business Combinations, and ASU 2017-01 and concluded that as substantially all of the fair value of the gross assets acquired is concentrated in a single identifiable asset or group of similar assets, the transaction did not meet the requirements to be accounted for as a business combination and therefore was accounted for as an asset acquisition. All consideration to be paid under the

License Agreement is contingent in nature and will be recognized when the respective contingency is resolved.

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 (the FAK Products) for all therapeutic, diagnostic and prophylactic uses in humans. The Company is solely responsible, at its expense, for the clinical development of the FAK 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 FAK 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 \$2.0 million in developmental milestones and up to an additional \$125.0 million based on the successful attainment of regulatory

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and commercial sales milestones. Pfizer is also eligible to receive high single to mid-double digit royalties on future net sales of the FAK Products. The Company's royalty obligations with respect to each FAK Product in each country begin on the date of first commercial sale of the FAK Product in that country, and end on the later of 10 years after the date of first commercial sale of the FAK 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 FAK 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.

## 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 Internal Revenue Service. Under the 401(k) Plan, the Company may make discretionary contributions as approved by the board of directors. The Company made approximate contributions to the 401(k) Plan of \$273,000, \$162,000, and \$295,000 for the years ended December 31, 2017, 2016, and 2015, respectively.

## 12. Reduction in force

In October 2015, the Company announced a reduction of workforce by approximately 50% to 20 full time employees. All affected employees received severance pay and outplacement assistance. As a result of the reduction in force and associated costs, the Company paid one-time severance and related costs of \$1.1 million. Of these one-time severance and related costs, approximately \$713,000 and approximately \$349,000 was paid during the years ended December 31, 2016 and 2015, respectively.

## 13. Quarterly financial information (unaudited, in thousands, except per share data)

	First Quarter Ended	Second Quarter Ended	Third Quarter Ended	Fourth Quarter Ended
	March 31, 2017	June 30, 2017	September 30, 2017	December 31, 2017
Operating expenses:				
Research and development	\$ 8,385	\$ 9,042	\$ 17,743	\$ 11,253
General and administrative	4,763	4,425	5,394	6,799
Total operating expenses	13,148	13,467	23,137	18,052
Loss from operations	(13,148)	(13,467)	(23,137)	(18,052)
Interest income	155	140	121	145
Interest expense	(12)	(109)	(110)	(328)
Net loss	\$ (13,005)	\$ (13,436)	\$ (23,126)	\$ (18,235)
Net loss per share —basic and diluted	\$ (0.35)	\$ (0.36)	\$ (0.61)	\$ (0.43)
Weighted-average number of common shares used in net loss per share —basic and diluted	36,992	36,992	37,630	(a) 42,027 (a)(b)

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	First Quarter Ended	Second Quarter Ended	Third Quarter Ended September	Fourth Quarter Ended
	March 31, 2016	June 30, 2016	30, 2016	December 31, 2016
Operating expenses:				
Research and development	\$ 4,179	\$ 4,492	\$ 4,216	\$ 6,892
General and administrative	4,255	4,217	3,843	4,908
Total operating expenses	8,434	8,709	8,059	11,800
Loss from operations	(8,434)	(8,709)	(8,059)	(11,800)
Interest income	140	140	137	145
Net loss	\$ (8,294)	\$ (8,569)	\$ (7,922)	\$ (11,655)
Net loss per share —basic and diluted	\$ (0.22)	\$ (0.23)	\$ (0.21)	\$ (0.32)
Weighted-average number of common shares used in net loss per share —basic and diluted	36,975	36,992	36,992	36,992

- (a) In the third and fourth quarters of 2017, the Company sold 2,853,753 and 2,183,126 shares of its common stock under the Company's at-the-market equity offering program, which resulted in net proceeds of \$14.1 million and \$9.0 million, respectively.
- (b) In December 2017, the Company closed an underwritten offering in which it sold 8,422,877 shares of its common stock at a price of \$2.97 per share, for aggregate proceeds, net of underwriting discounts and offering costs of \$24.7 million.

## 14. 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 carrying value of assets or liabilities as of the consolidated balance sheets date. The Company is not aware of any material subsequent events other than those disclosed above.