

Verastem, Inc.  
Form 10-K  
March 12, 2019  
Table of Contents

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, 2018

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 every Interactive Data File required to be submitted 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). Yes No

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§ 229.405 of this chapter) 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, a smaller reporting company or an emerging growth company. See definitions of "large accelerated filer," "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer Accelerated filer Non accelerated filer Smaller reporting company Emerging growth company

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, 2018 was \$503,007,333.

The number of shares outstanding of the registrant's common stock as of March 7, 2019 was 73,865,036.

#### DOCUMENTS INCORPORATED BY REFERENCE

Portions of the definitive proxy statement to be filed with the Securities and Exchange Commission pursuant to Regulation 14A relating to the Registrant's Annual General Meeting of Shareholders, to be held on May 14, 2019, will be incorporated by reference in this Form 10-K in response to Items 10, 11, 12, 13 and 14 of Part III. The definitive proxy statement will be filed with the Securities and Exchange Commission not later than 120 days after the registrant's fiscal year ended December 31, 2018.



Table of Contents

TABLE OF CONTENTS

PART I

<u>Item 1.</u>	<u>Business</u>	4
<u>Item 1A.</u>	<u>Risk Factors</u>	41
<u>Item 1B.</u>	<u>Unresolved Staff Comments</u>	72
<u>Item 2.</u>	<u>Properties</u>	73
<u>Item 3.</u>	<u>Legal Proceedings</u>	73
<u>Item 4.</u>	<u>Mine Safety Disclosures</u>	73

PART II

<u>Item 5.</u>	<u>Market for Registrant’s Common Equity, Related Stockholder Matters and Issuers Purchases of Equity Securities</u>	73
<u>Item 6.</u>	<u>Selected Financial Data</u>	75
<u>Item 7.</u>	<u>Management’s Discussion and Analysis of Financial Condition and Results of Operations</u>	76
<u>Item 7A.</u>	<u>Quantitative and Qualitative Disclosures About Market Risk</u>	94
<u>Item 8.</u>	<u>Consolidated Financial Statements and Supplementary Data</u>	94
<u>Item 9.</u>	<u>Changes in and Disagreements With Accountants on Accounting and Financial Disclosure</u>	94
<u>Item 9A.</u>	<u>Controls and Procedures</u>	94
<u>Item 9B.</u>	<u>Other Information</u>	97

PART III

<u>Item 10.</u>	<u>Directors, Executive Officers and Corporate Governance</u>	98
<u>Item 11.</u>	<u>Executive Compensation</u>	98
<u>Item 12.</u>	<u>Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters</u>	98
<u>Item 13.</u>	<u>Certain Relationships and Related Transactions, and Director Independence</u>	98
<u>Item 14.</u>	<u>Principal Accountant Fees and Services</u>	98

PART IV

<u>Item 15.</u>	<u>Exhibits and Financial Statement Schedules</u>	99
<u>Item 16.</u>	<u>Form 10-K Summary</u>	99

<u>EXHIBIT INDEX</u>	100
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<u>SIGNATURES</u>	104
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Table of Contents

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 and activity of our lead product, COPIKTRA and our Phosphoinositide 3-kinase (PI3K) and Focal Adhesion Kinase (FAK) programs generally, the potential commercial success of COPIKTRA, the anticipated adoption of COPIKTRA by patients and physicians, the structure of our planned and pending clinical trials, and the timeline and indications for clinical development, regulatory submissions and commercialization activities. The words “anticipate,” “believe,” “estimate,” “expect,” “intend,” “may,” “plan,” “predict,” “project,” “target,” “potential,” “will,” “would,” “could,” “should,” “continue” and other expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

Forward-looking statements are not guarantees of future performance and our actual results could differ materially from the results discussed in the forward-looking statements we make. Applicable risks and uncertainties include the risks and uncertainties, among other things, regarding: the commercial success of COPIKTRA in the United States; physician and patient adoption of COPIKTRA, including those related to the safety and efficacy of COPIKTRA; the uncertainties inherent in research and development of COPIKTRA, such as negative or unexpected results of clinical trials; whether and when any applications for COPIKTRA may be filed with regulatory authorities in any other jurisdictions; whether and when regulatory authorities in any other jurisdictions may approve any such other applications that may be filed for COPIKTRA, which will depend on the assessment by such regulatory authorities of the benefit-risk profile suggested by the totality of the efficacy and safety information submitted and, if approved, whether COPIKTRA will be commercially successful in such jurisdictions; our ability to obtain, maintain and enforce patent and other intellectual property protection for COPIKTRA and our other product candidates; the scope, timing, and outcome of any legal proceedings; decisions by regulatory authorities regarding labeling and other matters that could affect the availability or commercial potential of COPIKTRA; the fact that regulatory authorities in the U.S. or other jurisdictions, if approved, could withdraw approval; whether preclinical testing of our product candidates and preliminary or interim data from clinical trials will be predictive of the results or success of ongoing or later clinical trials; that the timing, scope and rate of reimbursement for our product candidates is uncertain; that third-party payors (including government agencies) may not reimburse for COPIKTRA; 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 COPIKTRA or our other product candidates will cause unexpected safety events, experience manufacturing or supply interruptions or failures, or result in unmanageable safety profiles as compared to their levels of efficacy; that COPIKTRA 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, CSPC Pharmaceutical Group Limited, Yakult Honsha Co. Ltd., or Infinity Pharmaceuticals, Inc. will fail to fully perform under the duvelisib license agreements; 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 chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) or indolent non-Hodgkin lymphoma (iNHL) in other jurisdictions; 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, 2018, 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.

Table of Contents

PART I

Item 1. Business

OVERVIEW

We are a biopharmaceutical company focused on developing and commercializing medicines to improve the survival and quality of life of cancer patients. Both our marketed product, COPIKTRA™ (duvelisib) capsules, and most advanced product candidate, defactinib, utilize a multi-faceted approach designed to treat cancers originating either in the blood or major organ systems. We are currently developing our product candidates in both preclinical and clinical studies as potential therapies for certain cancers, including leukemia, lymphoma, lung cancer, mesothelioma, ovarian cancer 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.

COPIKTRA is an oral inhibitor of phosphoinositide 3-kinase (PI3K), and the first approved dual inhibitor of PI3K-delta and PI3K-gamma, two enzymes known to help support the growth and survival of malignant B-cells. PI3K signaling may lead to the proliferation of malignant B-cells and is thought to play a role in the formation and maintenance of the supportive tumor microenvironment. COPIKTRA was approved by the U.S. Food & Drug Administration (FDA) on September 24, 2018 and is now indicated for the treatment of adult patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) after at least two prior therapies and relapsed or refractory follicular lymphoma (FL) after at least two prior systemic therapies. The indication in FL is approved under accelerated approval based on overall response rate. Continued approval for this FL indication may be contingent upon verification and description of clinical benefits in confirmatory trials.

We are also developing duvelisib for the treatment of multiple types of cancer, the most advanced of which is designed to treat patients with peripheral T-cell lymphoma (PTCL). The development of duvelisib in PTCL has been awarded Fast Track status by the FDA and a registration study is underway. During 2019, we plan to continue to further develop duvelisib through the initiation of a confirmatory study of patients with FL and other sponsored trials. We also plan to report interim data for several ongoing investigator sponsored studies (ISTs).

Our second product candidate, defactinib, is a targeted inhibitor of the Focal Adhesion Kinase (FAK) signaling pathway. FAK is a non-receptor tyrosine kinase encoded by the Protein Tyrosine Kinase-2 (PTK-2) gene that is involved in cellular adhesion and, in cancer, metastatic capability. Similar to COPIKTRA, defactinib is delivered orally and designed to be a potential therapy for patients to take at home under the advice of their physician. Defactinib is currently being investigated in combination with immunotherapeutic and other agents through ISTs. During 2019, we plan to report results from certain ongoing dose escalation combination studies involving this product.

## Table of Contents

### OUR FOCUS

We are focused on the development and commercialization of small molecules for optimized efficacy and safety – primarily as orally available drugs and drug candidates that are designed to treat various forms of cancer. Cancer is a group of diseases characterized by the uncontrolled growth and spread of abnormal cells. The American Cancer Society estimated that in the United States in 2018, approximately 1.7 million new cases of cancer were diagnosed and over 600,000 people died from the disease. Current treatments for cancer include surgery, radiation therapy, chemotherapy, hormonal therapy, immunotherapy, and targeted therapy. Notwithstanding years of intensive research and clinical use, these current treatments often fail to cure cancer. For example, 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 the cancer cells or effectively disrupt the tumor microenvironment, potentially resulting in eventual disease progression.

Accordingly, 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 U.S. annual incidence, based on 2018 estimates from the National Cancer Institute’s Surveillance, Epidemiology, and End Results Program (NCI; SEER), is that during the year there were approximately 75,000 new cases of indolent non-Hodgkin lymphoma (iNHL), 234,000 new cases of lung cancer and 55,000 cases of pancreatic cancer.

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 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.

Our commercial product, COPIKTRA, and product candidate, defactinib, are currently used to treat, and are being evaluated for the treatment of, certain types of hematologic and solid cancers, including CLL/SLL, iNHL, T-cell lymphoma, lung cancer, mesothelioma, ovarian cancer, pancreatic cancer, and other advanced cancers.

#### Chronic Lymphocytic Leukemia/Small Lymphocytic Lymphoma, 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 the number of new incidences of CLL/SLL was 4.3 per 100,000 men and women per year based on 2011-2015 cases and that the five-year relative survival rate from 2008 to 2014 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 has resulted in a significant advancement of 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.

The five-year relative survival rate from 2008 to 2014 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,

## Table of Contents

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 much as 70% of the indolent lymphomas reported in American and European clinical trials. 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.

Despite the advanced stage, median survival for patients 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 immediately, an approach referred to as "active surveillance" (also known as "watchful waiting"). Active treatment is often 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 upon initial diagnosis and treatment. 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, generally with decreasing responsiveness upon each additional relapse.

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. In advanced disease, there are now several targeted therapies available including COPIKTRA, Aliqopa and Zydelig, which have similar mechanisms of action. The use of an oral agent like COPIKTRA, particularly as a monotherapy that can be used in the general community physician's armamentarium, may hold significant value for 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 60% 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, 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 (during which patients receive their own stem cells) to some patients who had a positive response to their initial chemotherapy. Although promising, there is no firm clinical data to support that undergoing a transplant in this setting is better than

not undergoing a transplant.

6

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## Table of Contents

The potential of additional oral agents, either as a monotherapy or in combination with other anti-cancer agents, 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, with 50% of all cases occurring in women older than 65 years. The American Cancer Society estimates that in 2019 there will be approximately 22,530 new cases of ovarian cancer diagnosed and approximately 13,980 ovarian cancer-related deaths.

Most patients are treated with a combination of surgery, chemotherapy, targeted therapy and radiation therapy.

Surgery is often comprehensive, seeking 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 treatment 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

In 2018, the NCI estimated that pancreatic cancer was the eleventh most common cancer diagnosed in the United States and that the disease represented 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. 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 91% of patients dying within five years of their initial diagnosis based on the five-year relative survival rate from 2008 to 2014. 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 non-small cell lung cancer (NSCLC) are squamous cell carcinoma, large cell carcinoma, and adenocarcinoma. Although NSCLCs are associated with cigarette smoke, adenocarcinomas may be found in patients who have never smoked. As a class, NSCLCs are relatively insensitive to chemotherapy and radiation therapy compared with small cell lung cancer (SCLC). Lung cancer is the leading cause of cancer-related mortality in the United States. The five-year relative survival rate from 2008 to 2014 for patients with NSCLC was approximately 19%.

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 a 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 majority of patients.

7

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## Table of Contents

### Mesothelioma

Mesothelioma is a form of cancer that is most often caused by asbestos and affects the smooth lining of the chest, lungs, heart, and abdomen. Mesothelioma most often forms in the pleural cavity of the chest or into the abdomen as 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.

### OUR STRATEGY

COPIKTRA and defactinib 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:

- Continuing to support and maintain a commercial infrastructure in the United States for the marketing of COPIKTRA in approved and indicated hematologic malignancies as an oral monotherapy for patients needing additional lines of therapy following previous treatment.
- Expanding the indications in which COPIKTRA and defactinib may be used. In parallel with the CLL/SLL, iNHL, PTCL, NSCLC, ovarian cancer, pancreatic cancer and mesothelioma trials and studies that we are currently conducting, we plan to pursue additional disease indications to expand the potential of our product and product candidate.
- Advancing our product candidates through clinical development. We have ongoing clinical trials and studies of duvelisib and defactinib both as single agents and in combination with other agents in several hematologic and solid tumor indications.

## Table of Contents

- Collaborating 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. Additionally, we may seek third-party collaborations outside of the United States to continue to maximize the benefits of our product and product candidate to patients around the world.
- Considering the acquisition or in licensing of 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.
- Building and maintaining 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 the development of new therapies for the treatment of cancer.

### OUR PRODUCT AND PRODUCT CANDIDATE

Our pipeline product and product candidate currently consist of COPIKTRA, which is now commercially available in the United States, having been approved by the FDA for the treatment of certain hematologic malignancies in September 2018, and defactinib, which continues to be evaluated in the clinic for the treatment of a variety of cancer types.

#### COPIKTRA (duvelisib)

Our lead product, COPIKTRA (duvelisib), is the first approved oral, dual inhibitor of PI3K-delta and PI3K-gamma. COPIKTRA received approval from the FDA on September 24, 2018 for the treatment of adult patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) after at least two prior therapies and relapsed or refractory follicular lymphoma (FL), after at least two prior systemic therapies. The indication in FL is approved under accelerated approval based on overall response rate and continued approval for this indication may be contingent upon verification and description of clinical benefits in confirmatory trials.

The FDA approved labeling for COPIKTRA includes a boxed warning for four fatal and/or serious toxicities: infections, diarrhea or colitis, cutaneous reactions, and pneumonitis. Additionally, we have implemented an informational Risk Evaluation and Mitigation Strategy (REMS), as requested by the FDA, to support physicians in managing dosing and adverse reactions in their patients on COPIKTRA. In addition to the boxed warning, use of COPIKTRA is also associated with adverse reactions, which may require dose reduction, treatment delay or discontinuation of COPIKTRA. Warnings and precautions are provided in the package insert for infections, diarrhea or colitis, cutaneous reactions, pneumonitis, hepatotoxicity, neutropenia, and embryo-fetal toxicity. The most common adverse reactions (reported in  $\geq 20\%$  of patients) were diarrhea or colitis, neutropenia, rash, fatigue, pyrexia, cough, nausea, upper respiratory infection, pneumonia, musculoskeletal pain, and anemia.

The approval of COPIKTRA by the FDA was based on results obtained from two FDA clinical studies – DUO™ and DYNAMO™. 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 25 mg BID compared to ofatumumab, a monoclonal antibody treatment, administered to patients who have been diagnosed with CLL/SLL and whose disease is relapsed or refractory. A total of 319 patients were included in the study, of which 160 patients were treated with COPIKTRA

and 159 patients were treated with ofatumumab. 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

9

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Table of Contents

Progression-Free Survival (PFS). The FDA and European Medicines Agency (EMA) granted orphan drug designation to duvelisib for the treatment of CLL/SLL.

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 overall response rate (ORR) as assessed by an independent review committee (IRC) and according to the revised International Working Group (IWG) Criteria, which includes a change in target nodal lesions in combination with other measurements to determine response to treatment. The FDA and EMA granted orphan drug designation to duvelisib for the treatment of FL.

## THE COPIKTRA LABEL

The CLL/SLL indication for COPIKTRA is based on data from a subset of patients in the DUO trial who had received two or more prior lines of therapy. These 196 patients were the majority of patients enrolled in DUO. The sub-analysis data included in the COPIKTRA label resulted in a median PFS by central review in this population of 16.4 months for COPIKTRA vs. 9.1 months for ofatumumab with a Standard Error of 2.1 and 0.5 months, respectively. This equates to a hazard ratio of 0.4, with a Standard Error of 0.2; or a 60% reduction in the risk of progression or death. Additionally, COPIKTRA achieved a 78% ORR, compared to 39% for ofatumumab – a 39% difference, with a Standard Error of 6.4%.

## Efficacy in CLL/SLL After at Least Two Prior Therapies (DUO)

Outcome per IRC	COPIKTRA	Ofatumumab
	N = 95	N=101
PFS		
Number of events, n (%)	55 (58%)	70 (69%)
Progressive disease	44	62
Death	11	8
Median PFS (SE), months a	16.4 (2.1)	9.1 (0.5)
Hazard Ratio (SE), b COPIKTRA/ofatumumab	0.40 (0.2)	
Response Rate		
ORR n (%) c	74 (78%)	39 (39%)
CR	0 (0%)	0 (0%)
PR	74 (78%)	39 (39%)
Difference in ORR, % (SE)	39% (6.4)	

Abbreviations: CI = confidence interval; CR = complete response; IRC = Independent Review Committee; PFS = progression-free survival; PR = partial response; SE = standard error

a Kaplan-Meier estimate

b Standard Error of  $\ln(\text{hazard ratio}) = 0.2$

c IWCLL or revised IWG response criteria, with modification for treatment-related lymphocytosis

Table of Contents

Kaplan-Meier Curve of PFS per IRC In Patients with at Least 2 Prior Therapies (DUO)

11

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Table of Contents

Overall Response Rate (ORR) per IRC (DUO)

Lymph Node Response Rate (LNRR) per IRC (DUO)

The primary data in support of the accelerated approval in FL by the FDA in the United States was derived from updated results for the subset of follicular lymphoma patients in the DYNAMO study. This subset of data was comprised of a pre-treated double refractory patient population with a median of 3 prior lines of therapy. In this patient population, treatment with COPIKTRA resulted in a 42% overall response rate, with a 95% confidence interval between 31% and 54%, and a maximum duration of response up to nearly 3 and a half years as of the last data cut-off. Based on this data, and an unmet need for additional therapy options in FL, COPIKTRA is now indicated for the treatment of U.S. patients with relapsed or refractory follicular lymphoma after at least two prior systemic therapies.

Table of Contents

## Efficacy in Patients with Relapsed or Refractory FL (DYNAMO)

Endpoint	FL
	N = 83
ORR, N (%) a	35 (42%)
95% CI	(31, 54)
CR, n (%)	1 (1%)
PR, n (%)	34 (41%)
Duration of response	
Range, months	0.0+ to 41.9+
Patients maintaining response at 6 months, n/N (%)	15/35 (43%)
Patients maintaining response at 12 months, n/N (%)	6/35 (17%)

Abbreviations: CI = confidence interval; CR = complete response; IRC = Independent Review Committee; ORR = overall response rate; PR = partial response

a Per IRC according to Revised International Working Group criteria

+ Denotes censored observation

The primary safety data in support of the COPIKTRA label comes from a pooled safety analysis conducted in 442 patients treated with COPIKTRA at the recommended starting dose of 25 mg BID. The results of this analysis are consistent with the data seen in the full DYNAMO and DUO studies.

Most Common Adverse Reactions ( $\geq 10\%$  Grade  $\geq 3$  or  $\geq 20\%$  Any Grade) in Patients with B-cell Malignancies Receiving COPIKTRA

Adverse Reactions	COPIKTRA 25 mg BID (N = 442)	
	Grade $\geq$ 3	Any Grade
	n (%)	n (%)
Neutropenia †	132 (30%)	151 (34%)
Diarrhea or colitis † <sup>a</sup>	101 (23%)	222 (50%)
Pneumonia † <sup>b</sup>	67 (15%)	91 (21%)
Anemia †	48 (11%)	90 (20%)
Rash † <sup>c</sup>	41 (9%)	136 (31%)
Fatigue †	22 (5%)	126 (29%)
Pyrexia	7 (2%)	115 (26%)
Musculoskeletal pain †	6 (1%)	90 (20%)
Nausea †	4 (<1%)	104 (24%)
Cough †	2 (<1%)	111 (25%)
Upper respiratory tract infection †	2 (<1%)	94 (21%)

† Grouped term for reactions with multiple preferred terms

<sup>a</sup> Diarrhea or colitis includes the preferred terms: colitis, enterocolitis, colitis microscopic, colitis ulcerative, diarrhea, diarrhea hemorrhagic

<sup>b</sup> Pneumonia includes the preferred terms: All preferred terms containing "pneumonia" except for "pneumonia aspiration"; bronchopneumonia, bronchopulmonary aspergillosis

<sup>c</sup> Rash includes the preferred terms: dermatitis (including allergic, exfoliative, perivascular), erythema (including multiforme), rash (including exfoliative, erythematous, follicular, generalized, macular & papular, pruritic, pustular), toxic epidermal necrolysis and toxic skin eruption, drug reaction with eosinophilia and systemic symptoms, drug eruption, Stevens-Johnson syndrome

Serious adverse reactions were reported in 289 patients (65%). The most frequent serious adverse reactions that occurred were infection (31%), diarrhea or colitis (18%), pneumonia (17%), rash (5%) and pneumonitis (5%).

Table of Contents

THE DUO AND DYNAMO STUDIES

As discussed above, COPIKTRA's indicated label for the treatment of CLL/SLL and FL patients was derived from subsets of the complete datasets from DUO and DYNAMO, respectively. Those complete results have subsequently been published in the public domain, as discussed below.

THE DUO STUDY

The results from the DUO study were presented at the 2017 Annual Meeting of the American Society for Hematology conference (ASH 2017) and published in the journal Blood in December 2018 (volume 132). 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 IRC using modified international workshop on CLL (iwCLL) or revised 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.

Median PFS per IRC

\*Flinn et al., ASH 2017

Similar efficacy results for duvelisib were 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.

Table of Contents

Median PFS per IRC for del[17p] Subpopulation

\*Flinn et al., ASH 2017

Median PFS per Investigator Assessment

\*Flinn et al., ASH 2017

15

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Table of Contents

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 ( $p = 0.0182$ ).

\*Flinn et al., ASH 2017

16

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## Table of Contents

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.

Following prolonged exposure in DUO, 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 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.

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

## THE DYNAMO STUDY

Similarly, 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). Eighty-three percent of patients had a reduction of target nodal lesions in lymph nodes.

Table of Contents

\*Adapted from Flinn et al., ASH 2016

\*Flinn et al., ASH 2016

Duvelisib demonstrated a consistent and manageable safety profile with appropriate risk mitigation in the DYNAMO study. 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).

## Table of Contents

Based largely on the clinical results of the DUO and DYNAMO studies, the National Comprehensive Cancer Network (NCCN) added COPIKTRA to the Clinical Practice Guidelines in Oncology (NCCN Guidelines), the standard physician resource for determining the appropriate course of treatment for patients, for CLL/SLL, FL and MZL. We believe these updated guidelines will increase awareness for COPIKTRA and help health care providers make informed decisions for patients battling these difficult to treat advanced cancers.

In addition, duvelisib is being evaluated as an investigational compound in clinical trials, both as a monotherapy and in combination with other anti-cancer agents, in hematologic or solid tumor malignancies. The safety and efficacy of these investigational uses of duvelisib have not yet been evaluated by the FDA or any other health authority for marketing authorization.

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

In a Phase 1 study published in *Blood* in February 2018, the ORR in patients with PTCL treated with duvelisib monotherapy (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 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 (PRIMO) evaluating the efficacy and safety of duvelisib in patients with relapsed or refractory PTCL. This study is currently being conducted in the United States and we expect to expand the study to the European Union, Japan and potentially other territories.

### 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 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.

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

## Table of Contents

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.

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.

## 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.

20

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## Table of Contents

### 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.

#### PI3K inhibition program

As previously discussed, we are currently marketing and continuing to develop the PI3K inhibitor COPIKTRA (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. We have applied for patent term extension for US 8,193,182, which, if granted, will extend the term of the portion covering duvelisib to 2033. 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 also currently developing the FAK inhibitor defactinib.

We have exclusively licensed a portfolio of patent applications owned by Pfizer, Inc. (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 enzymes (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

21

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## Table of Contents

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 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. As stated above, we have applied for patent term extension for US 8,193,182, which, if granted, will extend the term of the portion covering duvelisib to 2033.

## 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 maintained a portion of the financial responsibility for the shutdown of certain other clinical studies. We are obligated to use diligent efforts to develop and commercialize a product in an oncology indication containing duvelisib. As previously discussed, COPIKTRA was approved by the FDA on September 24, 2018 and is now indicated for the treatment of adult patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) after at least two prior therapies and relapsed or refractory follicular lymphoma (FL) after at least two prior systemic therapies. 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 a New Drug Application (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, which was paid in cash by us to Infinity in November 2018.

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

Table of Contents

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 us if litigation were to arise, with any such reductions capped at 50% of the amounts otherwise payable during the applicable royalty payment period.

## Table of Contents

### Yakult Honsha Co., Ltd.

On June 5, 2018, we entered into a license and collaboration agreement (the Yakult Agreement) with Yakult Honsha Co., Ltd. (Yakult), under which we granted exclusive rights to Yakult to develop and commercialize products containing duvelisib in Japan for the treatment, prevention, palliation or diagnosis of all oncology indications in humans or animals.

Under the terms of the Yakult Agreement, Yakult received an exclusive right to develop and commercialize products containing duvelisib in Japan under mutually agreed development and commercialization plans at its own cost and expense. Yakult also received certain limited manufacturing rights in the event that we are unable to manufacture or supply sufficient quantities of duvelisib or products containing duvelisib to Yakult during the term of the Yakult Agreement. We retained all rights to duvelisib outside of Japan.

Yakult paid us an upfront, non-refundable payment of \$10.0 million in June 2018. We are also entitled to receive aggregate payments of up to \$90.0 million if certain development, regulatory and commercial milestones are successfully achieved. Yakult is obligated to pay us a double-digit royalty on net sales of products containing duvelisib in Japan, subject to reduction in certain circumstances, and to fund certain global development costs related to worldwide clinical trials conducted by us in which Yakult has opted to participate (Global Clinical Trials) on a pro-rata basis.

Unless earlier terminated by either party, the Yakult Agreement will expire upon the fulfillment of Yakult's royalty obligations to us for the sale of any products containing duvelisib in Japan, which royalty obligations expire, on a product-by-product basis, upon the last to occur of (a) expiration of valid claims covering such product, (b) expiration of regulatory exclusivity for such product or (c) 10 years from first commercial sale of such product. Yakult may terminate the Yakult Agreement in its entirety at any time with 180 days' written notice. Either party may terminate the Yakult Agreement in its entirety with 60 days' written notice for the other party's material breach if such party fails to cure the breach. We may terminate the Yakult Agreement if (i) Yakult fails to use commercially reasonable efforts to develop and commercialize products containing duvelisib in Japan or (ii) Yakult challenges any patent licensed by us to Yakult under the Yakult Agreement. Either party may terminate the Yakult Agreement in its entirety upon certain insolvency events involving the other party.

### CSPC Pharmaceutical Group Limited (CSPC)

On September 25, 2018, we entered into a license and collaboration agreement with CSPC (the CSPC Agreement), under which we granted exclusive rights to CSPC to develop and commercialize products containing duvelisib in the People's Republic of China (China), Hong Kong, Macau and Taiwan (collectively, the CSPC Territory) for the treatment, prevention, palliation or diagnosis of all oncology indications in humans.

Under the terms of the CSPC Agreement, CSPC received an exclusive right to develop and commercialize products containing duvelisib in the CSPC Territory under mutually agreed upon development and commercialization plans at its own cost and expense. CSPC also received certain limited manufacturing rights in the event that we are unable to manufacture or supply sufficient quantities of duvelisib or products containing duvelisib to CSPC during the term of the CSPC Agreement. We retained all rights to duvelisib outside of the CSPC Territory.

CSPC paid us an aggregate upfront, non-refundable payment of \$15.0 million, \$5.0 million of which had already been paid by CSPC as a non-refundable exclusivity fee. We are also entitled to receive aggregate payments of up to \$160.0 million if certain development, regulatory and commercial milestones are successfully achieved. CSPC is obligated to pay us a double-digit royalty on net sales of products containing duvelisib in the CSPC Territory, subject to reduction in certain circumstances, and to fund certain global development costs related to worldwide clinical trials conducted

by us in which CSPC has opted to participate (Global Clinical Trials) on a pro-rata basis.

24

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## Table of Contents

Unless earlier terminated by either party, the CSPC Agreement will expire upon the fulfillment of CSPC's royalty obligations to us for the sale of any products containing duvelisib in the CSPC Territory, which royalty obligations expire, on a product-by-product basis, upon the last to occur of (a) expiration of valid claims covering such product, (b) expiration of regulatory exclusivity for such product or (c) 10 years from first commercial sale of such product. CSPC may terminate the CSPC Agreement in its entirety at any time with 180 days' written notice. Either party may terminate the CSPC Agreement in its entirety with 60 days' written notice for the other party's material breach if such party fails to cure the breach. We may terminate the CSPC Agreement if (i) CSPC fails to use commercially reasonable efforts to develop and commercialize products containing duvelisib in the CSPC Territory or (ii) CSPC challenges any patent licensed by us to CSPC under the CSPC Agreement. Either party may terminate the CSPC Agreement in its entirety upon certain insolvency events involving the other party.

### 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 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



## Table of Contents

acquiring technologies complementary to, or necessary for, our programs. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

The key competitive factors affecting the success of all 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, immunotherapy, 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 the PI3K signaling pathway:

- Gilead Sciences, Inc., which has received approval from the FDA of idelalisib for the treatment of patients with CLL, SLL, or FL, and which we believe has completed a Phase 1b clinical trial of acalisib (GS-9820);
- Bayer AG, which has received approval from the FDA of copanlisib for the treatment of patients with relapsed FL;

Table of Contents

- Adlai Noryte, which we believe has completed a Phase 2 clinical trial of buparlisib;
- AstraZeneca, which we believe is conducting Phase 1 and Phase 2 clinical trials of ACP-319;
- TG Therapeutics, Inc., which we believe is conducting multiple clinical trials of TGR-1202;
- Incyte Corporation, which we believe is conducting a Phase 2 clinical trial of INCB050465;
- MEI Pharma, which we believe is conducting Phase 1b and Phase 2 clinical trials of ME-401; and
- Rhizen Pharmaceuticals, which we believe is conducting Phase 2 clinical trials for tenalisib.

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 drug candidates 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 acalabrutinib (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.

## Table of Contents

### MANUFACTURING

We contract with third parties for the manufacture of COPIKTRA for commercial and clinical use and for the manufacture of our product candidates for preclinical studies and clinical trials, and we intend to continue to do so in the future. We currently work with one contract manufacturing organization (CMO) for the production of duvelisib drug substance, one CMO for the production of oral drug product, and one CMO for the final commercial and clinical packaging. We have long-term supply agreements in place with each of these CMOs. We are currently evaluating a second source supplier program for the manufacture and packaging of duvelisib. For defactinib, we have one CMO for the manufacture of drug product, one CMO for the production of drug substance, and one CMO for drug packaging. We obtain drug product or substance from these manufacturers on a purchase order basis. We may elect to pursue relationships with other CMOs for manufacturing clinical supplies for later-stage clinical trials and for commercialization. We do not own or operate, and currently have no plans to establish, any manufacturing facilities. We have personnel with pharmaceutical development and manufacturing experience who are responsible for the relationships with our CMOs.

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 the 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.

### COMMERCIAL STRATEGY

We intend to develop and commercialize our drugs in the U.S., Canada and the European Union alone or with partners, and expect to rely on partners to develop and commercialize our drugs in other territories throughout the world. On September 24, 2018, our first commercial product, COPIKTRA, was approved by the FDA for the treatment of patients with hematologic cancers including CLL/SLL and FL. We sell COPIKTRA to a limited number of specialty pharmacies and specialty distributors in the United States. These customers subsequently resell COPIKTRA either directly to patients, or to community hospitals or oncology clinics with in-office dispensaries who in turn distribute COPIKTRA to patients. In the U.S., our sales team promotes our commercial product for its approved indications through direct field contact with physicians, hospitals, clinics and other healthcare providers.

None of our product candidates have received regulatory approval for commercial sale in territories outside of the United States. As set forth above, we have entered into agreements with third-party partners for the development and commercialization of duvelisib in territories outside of the United States and have agreed to manufacture or supply quantities of our product candidate in conjunction with these efforts. We continue to evaluate opportunities and potential partnerships to develop and commercialize duvelisib in territories outside the United States. In executing these arrangements, our goal is to retain significant worldwide oversight over the development process and commercialization of our products by playing an active role in their commercialization or finding partners who share our vision, values, culture and processes.

### APPLICABLE LAWS AND 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

## Table of Contents

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, 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

## Table of Contents

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 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

30

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## Table of Contents

application should be approved. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

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

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

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

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

### Fast track designation

The FDA is required to facilitate the development and expedite the review of drugs that are intended for the treatment of a serious or life threatening condition for which there is no effective treatment and which demonstrate the potential to address unmet medical needs for the condition. Under the fast track program, the sponsor of a new drug candidate may request the FDA to designate the product for a specific indication as a fast track product concurrent with or after the filing of the IND for the product candidate. The FDA must determine if the product candidate qualifies for fast track designation within 60 days after receipt of the sponsor's request.

In addition to other benefits, such as the ability to use surrogate endpoints and have greater interactions with the FDA, the FDA may initiate review of sections of a fast track product's NDA before the application is complete. This rolling review is available if the applicant provides and the FDA approves a schedule for the submission of the remaining information and the applicant pays applicable user fees. However, the FDA's time period goal for reviewing a fast track application does not begin until the last section of the NDA is submitted. In addition, the fast track designation

may be withdrawn by the FDA if the FDA believes that the designation is no longer supported by data emerging in the clinical trial process.

31

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## Table of Contents

### 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 one or more 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 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

32

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## Table of Contents

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 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.



## Table of Contents

### 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.

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;



## Table of Contents

- 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

We are 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 sales of any of our products. 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 could be subject to challenge.

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,

35

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## Table of Contents

clinical trials, marketing authorization, commercial sales and distribution of our products. Regardless of our current FDA approval or any future FDA approvals we may obtain 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 new drug products. Sales of COPIKTRA or any other 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.

Within the United States, FDA-approved drugs could potentially be covered by various government health benefit programs as well as purchased by government agencies. The participation in such programs or the sale of products to such agencies is subject to regulation. The marketability of any of our approved products may suffer if the government and third-party payors fail to provide adequate coverage and reimbursement.

Medicaid is a joint federal and state program that is administered by the states for low income and disabled beneficiaries. Under the Medicaid Drug Rebate Program, participating manufacturers are required to pay a rebate for each unit of product reimbursed by the state Medicaid programs. The amount of the rebate for each product is set by law and may be subject to an additional discount if certain pricing increases more than inflation.

Medicare is a federal program that is administered by the federal government that covers individuals age 65 and over as well as those with certain disabilities. Oral drugs may be covered under Medicare Part D. Medicare Part D provides coverage to enrolled Medicare patients for self-administered drugs (i.e., drugs that do not need to be injected or otherwise administered by a physician). Medicare Part D is administered by private prescription drug plans approved by the U.S. government and each drug plan establishes its own Medicare Part D formulary for prescription drug coverage and pricing, which the drug plan may modify from time-to-time. The prescription drug plans negotiate pricing with manufacturers and may condition formulary placement on the availability of manufacturer discounts. Since 2011, manufacturers with marketed brand name drugs have been required to provide a 50% discount the negotiated price for on brand name prescription drugs utilized by Medicare Part D beneficiaries when those beneficiaries reach the coverage gap in their drug benefits, and, beginning in 2019, that discount increased to 70%.

Drug products are subject to discounted pricing when purchased by federal agencies via the Federal Supply Schedule (FSS). FSS participation is required for a drug product to be covered and reimbursed by certain federal agencies and for coverage under Medicaid, Medicare Part B and the Public Health Service (PHS) pharmaceutical pricing program. FSS pricing is negotiated periodically with the Department of Veterans Affairs. FSS pricing is intended not to exceed the price that a manufacturer charges its most-favored non-federal customer for its product.

## Table of Contents

In addition, prices for drugs purchased by the Veterans Administration, Department of Defense (including drugs purchased by military personnel and dependents through the TRICARE retail pharmacy program), Coast Guard, and PHS are subject to a cap on pricing (known as the “federal ceiling price”) and may be subject to an additional discount if pricing increases more than the rate of inflation.

To maintain coverage of drugs under the Medicaid Drug Rebate Program, manufacturers are required to extend discounts to certain purchasers under the PHS pharmaceutical pricing program. Purchasers eligible for discounts include hospitals that serve a disproportionate share of financially needy patients, community health clinics and other entities that receive health services grants from the PHS.

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 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 COPIKTRA or any other products for which we have or will receive regulatory approval for commercial sale may suffer if the government and third party payors fail to provide adequate coverage and reimbursement. In addition, there is an increasing emphasis on managed care in the United States 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 a product, 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. 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.

## Table of Contents

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 Healthcare 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 Healthcare Reform Act. For example, tax reform legislation was enacted at the end of 2017 that eliminates the tax penalty for individuals who do not maintain mandated health insurance coverage beginning in 2019. In a May 2018 report, the Congressional Budget Office estimated that, compared to 2018, the number of uninsured will increase by 3 million in 2019 and 6 million in 2028, in part due to the elimination of the individual mandate.

The Healthcare Reform Act has also been subject to judicial challenge. In December 2018, a federal district court, in a challenge brought by a number of state attorneys general, found the Healthcare Reform Act unconstitutional in its entirety because, once Congress repealed the individual mandate provision, there was no longer a basis to rely on Congressional taxing authority to support enactment of the law. Pending appeals, which could take some time, the Healthcare Reform Act is still operational in all respects.

There have also been efforts by government officials or legislators to implement measures to regulate drug pricing 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. Specifically, at the federal level, for example, in May 2018, President Trump and the Secretary of the Department of Health and Human Services released a “blueprint” to lower prescription drug prices and out-of-pocket costs. Certain proposals in the blueprint, and related drug pricing measures proposed since the blueprint, could cause significant operational and reimbursement changes for the pharmaceutical industry. In October 2018, the Centers for Medicare & Medicaid Services, or CMS, solicited public comments on potential changes to payment for certain Medicare Part B drugs, including reducing the Medicare payment amount for selected Medicare Part B drugs to more closely align with international drug prices. As another example, in November of 2018, CMS issued an advance notice of proposed rulemaking that proposed revisions to Medicare Part D to support health plans’ negotiation of lower drug prices with manufacturers and reduce health plan members’ out-of-pocket costs. The HHS Office of Inspector General also issued a proposed rule in February of 2019 that would revise the federal anti-kickback statute to limit protection for discounts offered by pharmaceutical manufacturers to pharmacy benefit managers (“PBMs”), Medicare Part D plans, and Medicaid managed care plans that are not reflected in the price charged to the patient at the pharmacy counter and to provide protection only for certain types of service fees paid by pharmaceutical manufacturers to PBMs.

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 December 31, 2018, we had 169 full time equivalent employees, including a total of 17 employees with M.D. or Ph.D. degrees, and 3 part-time employees. Of the full time employees, 33 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.

38

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Table of Contents

## 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, 2019.

Name	Age	Position
Robert Forrester	55	President, Chief Executive Officer
Daniel Paterson	57	Chief Operating Officer
Robert Gagnon	44	Chief Financial Officer
Joseph Lobacki	60	Chief Commercial Officer
Steven Bloom	58	Chief Strategy Officer

Robert Forrester, age 55, 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 Pharmaceutical Group, Inc. from 2000 to 2003. He earned his LL.B. from Bristol University in England.

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.

Robert Gagnon, age 44, has served as our Chief Financial Officer since August 2018. Prior to joining us, Mr. Gagnon served as the Chief Financial Officer for Harvard Bioscience, Inc. from November 2013 to August 2018. From 2012 through 2013, Mr. Gagnon served as the Executive Vice President, Chief Financial Officer and Treasurer at Clean Harbors, Inc. Mr. Gagnon's prior experience includes serving as Chief Accounting Officer and Controller at Biogen Idec, Inc., as well as a variety of senior positions at Deloitte & Touche, LLP, and PriceWaterhouseCoopers, LLP.

Joseph Lobacki, age 60, 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.

Steven Bloom, age 58, 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.



Table of Contents

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.

Table of Contents

ITEM 1A. Risk Factors.

Risks Related to the Commercialization of COPIKTRA and Development of Our Product Candidates

We are dependent on the commercial success of COPIKTRA.

A majority of our time, resources and effort are focused on the commercialization of COPIKTRA in the United States. While we expect to continue to expend significant time, resources and effort on the development of our other product candidates, they are in earlier stages of development and subject to the risks of failure inherent in developing drug products.

Our ability to successfully commercialize COPIKTRA will depend on, among other things, our ability to:

- maintain commercial manufacturing arrangements with CMOs;
- produce, through a validated process, sufficient quantities and inventory of COPIKTRA to meet demand;
- build and maintain internal sales, distribution and marketing capabilities sufficient to generate commercial sales of COPIKTRA;
- secure widespread acceptance of our product from physicians, health care payors, patients and the medical community;
- properly price and obtain coverage and adequate reimbursement of COPIKTRA by governmental authorities, private health insurers, managed care organizations and other third-party payors;
- maintain compliance with ongoing FDA labeling, packaging, storage, advertising, promotion, recordkeeping, safety and other post-market requirements;
- manage our growth and spending as costs and expenses increase due to commercialization; and
- establish and maintain collaborations with third parties for the commercialization of COPIKTRA in countries outside the United States, and such collaborators' ability to obtain regulatory and reimbursement approvals in such countries.

There are no guarantees that we will be successful in completing these tasks. In addition, we have begun, and will need to continue investing substantial financial and management resources to build out our commercial infrastructure and to recruit and train sufficient additional qualified marketing, sales and other personnel in support of our sales of COPIKTRA.

Sales of COPIKTRA may be slow or limited for a variety of reasons including competing therapies or safety issues. If COPIKTRA is not successful in gaining broad commercial acceptance, our business would be harmed.

Any sales of COPIKTRA will be dependent on several factors including our ability to educate and increase physician awareness of the benefits and cost-effectiveness of COPIKTRA relative to competing therapies. The degree of market acceptance of COPIKTRA among physicians, patients, health care payors and the medical community will depend on a number of factors, including:

- acceptable evidence of safety and efficacy;
- relative convenience and ease of administration;
- prevalence and severity of any adverse side effects;
- availability of alternative treatments;
- pricing and cost effectiveness;
- effectiveness of our sales and marketing capability and strategies;
- ability to obtain sufficient third-party coverage and reimbursement;
- changes in the standard of care for the targeted indications for COPIKTRA;

- warnings and limitations, including the boxed warning related to the risks of infections, diarrhea or colitis, cutaneous reactions, and pneumonitis, contained in the approved labeling for COPIKTRA;
- safety concerns with similar products marketed by others;

## Table of Contents

- the prevalence and severity of any side effects as a result of treatment with COPIKTRA;
- our ability to comply with FDA post-marketing requirements imposed upon COPIKTRA, including conducting and completing a confirmatory clinical trial in patients with relapsed or refractory follicular lymphoma that verifies and isolates the benefits of COPIKTRA; and
- the actual market-size for COPIKTRA, which may be larger or smaller than expected.

In addition, COPIKTRA will be subject to continual review by the FDA, and we cannot assure you that newly discovered or developed safety issues will not arise. With the use of any newly marketed drug by a wider patient population, serious adverse events may occur from time to time that initially do not appear to relate to the drug itself. Any safety issues could cause us to suspend or cease marketing COPIKTRA, cause us to modify how we market COPIKTRA, subject us to substantial liabilities and adversely affect our revenues and financial condition. In the event of a withdrawal of COPIKTRA from the market, our revenues would decline significantly and our business would be seriously harmed and could fail. We additionally may experience significant fluctuations in sales of COPIKTRA from period to period and, ultimately, we may never generate sufficient revenues from COPIKTRA to reach or maintain profitability or sustain our anticipated operations.

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, including COPIKTRA. If we are unable to obtain marketing approval for or successfully commercialize 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 COPIKTRA, for which we are conducting clinical trials in multiple indications. We received FDA approval for COPIKTRA for the treatment of adult patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) after at least two prior therapies and were granted accelerated approval of COPIKTRA for the treatment of adult patients with relapsed or refractory follicular lymphoma (FL) after at least two prior systemic therapies. Our ability to generate product revenues will depend heavily on the successful commercialization of COPIKTRA and development of our other 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 future product candidates, including pricing approvals where required;
- establishing and maintaining 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 and maintaining 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.



## Table of Contents

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.

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, 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 our 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;





Table of Contents

- 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 or not obtain marketing approval for our product candidates;
- 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;
- 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.



## Table of Contents

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 commercialization of COPIKTRA or development of our other product candidates, we may need to abandon or limit the commercialization of COPIKTRA and abandon or limit our development of some of our other product candidates.

The FDA approved COPIKTRA with labeling that includes a boxed warning for four fatal and/or serious toxicities: infections, diarrhea or colitis, cutaneous reactions, and pneumonitis. As a requirement of the FDA's approval, we are implementing an informational REMS to provide appropriate dosing and safety information to better support physicians in managing their patients on COPIKTRA. In addition to the boxed warning, use of COPIKTRA is also associated with adverse reactions, which may require dose reduction, treatment delay or discontinuation of COPIKTRA. Warnings and precautions are provided for infections, diarrhea or colitis, cutaneous reactions, pneumonitis, hepatotoxicity, neutropenia, and embryo-fetal toxicity. The most common adverse reactions (reported in  $\geq 20\%$  of patients) were diarrhea or colitis, neutropenia, rash, fatigue, pyrexia, cough, nausea, upper respiratory infection, pneumonia, musculoskeletal pain, and anemia.

Our other product candidates are in various stages of clinical development and their risk of failure is high. It is impossible to predict when or if our other 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. 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

## Table of Contents

that a serious adverse effect or unacceptable side effect was not drug related.

For COPIKTRA, if we or others identify previously unknown side effects or if known side effects are more frequent or severe than in the past, then:

- sales of COPIKTRA may be adversely affected;
- regulatory approvals for COPIKTRA may be restricted or withdrawn;
- we may decide to, or be required to, send product warning letters or field alerts to physicians, pharmacists and hospitals;
- additional non-clinical or clinical studies, changes in labeling or changes to manufacturing processes, specifications and/or facilities may be required; and
- government investigations or lawsuits, including class action suits, may be brought against us.

Any of the above occurrences would harm or prevent sales of COPIKTRA, increase our expenses and impair our ability to successfully commercialize COPIKTRA. Furthermore, as COPIKTRA is commercially available, it may be used in a wider population and in a less rigorously controlled environment than in clinical studies. As a result, regulatory authorities, healthcare practitioners, third-party payors or patients may perceive or conclude that the use of COPIKTRA is associated with previously unknown serious adverse effects, undermining our commercialization efforts.

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 relatively unproven, and we do not know whether we will be able to develop any products of significant commercial value.

We are commercializing COPIKTRA and developing duvelisib in other indications and other 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 still uncertainty about whether COPIKTRA 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.

## Table of Contents

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 commercializing COPIKTRA and conducting clinical trials for other 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 candidates 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 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.

## Table of Contents

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, including COPIKTRA, 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 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 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 COPIKTRA and our other product candidates and will face competition with respect to any product



## Table of Contents

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 COPIKTRA and 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 commercializing COPIKTRA and developing our other 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 COPIKTRA and our other product candidates, if approved, 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 products or product candidates of our competitors demonstrate serious adverse side effects or are determined to be ineffective in clinical trials, the commercialization of COPIKTRA and the development of our other product candidates could be negatively impacted.

COPIKTRA and any future product candidates that we commercialize may become subject to unfavorable pricing regulations or third-party coverage and reimbursement policies, which would harm our business.

In both domestic and foreign markets, sales of COPIKTRA and any product candidates that may receive marketing approval in the future will depend, in part, on favorable pricing as well as the availability of coverage and amount of reimbursement by third party payors, including governments and private health plans. Substantial uncertainty exists regarding coverage and reimbursement by third party payors of newly approved health care products.

Outside the United States, some countries require approval of the sale price of a drug before the product can be marketed. In many such 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 COPIKTRA and other product candidates, even if those product candidates obtain marketing approval.

Cost containment is a key trend in the United States and elsewhere. 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

## Table of Contents

available for COPIKTRA or any other 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, COPIKTRA or any other product candidate for which we obtain marketing approval. If coverage and reimbursement are not available or reimbursement is available only to limited levels, we may not be able to successfully commercialize COPIKTRA or any other product candidate for which we may obtain marketing approval.

If we fail to comply with our reporting and payment obligations under the Medicaid Drug Rebate Program or other governmental pricing programs in the U.S., we could be subject to additional reimbursement requirements, penalties, sanctions and fines which could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

With the approval of COPIKTRA, we now participate in the Medicaid Drug Rebate Program, Medicare Coverage Gap Discount Program and a number of other federal and state government pricing programs in the U.S. in order to obtain coverage for the product by certain government healthcare programs. These programs generally require us to pay rebates or provide discounts to certain private purchasers or government payors in connection with our products when dispensed to beneficiaries of these programs. In some cases, such as with the Medicaid Drug Rebate Program, the rebates are based on pricing and rebate calculations that we report on a monthly and quarterly basis to the government agencies that administer the programs. The terms, scope and complexity of these government pricing programs change frequently. We may also have reimbursement obligations or be subject to penalties if we fail to provide timely and accurate information to the government, pay the correct rebates or offer the correct discounted pricing. Changes to the price reporting or rebate requirements of these programs would affect our obligations to pay rebates or offer discounts. Responding to current and future changes may increase our costs and the complexity of compliance, will be time-consuming, and could have a material adverse effect on our results of operations.

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

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 from any sales of COPIKTRA or if we commercially sell any other products 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 COPIKTRA or any other 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 commercialize COPIKTRA and any future product candidates or if we initiate additional clinical trials in the United States and around the world. 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

50

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## Table of Contents

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 COPIKTRA program, our business could be adversely affected.

Our license agreement with Infinity for COPIKTRA 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 COPIKTRA on our financial results relating to numerous matters, including:

- the cost of development and commercialization of COPIKTRA; 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 COPIKTRA may not be realized or be of the magnitude expected.

### Risks Related to Our Financial Position and Need for Additional Capital

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

Since inception, we have incurred significant operating losses. As of December 31, 2018, we had an accumulated deficit of \$375.6 million. To date, we have generated minimal product revenues and have financed our operations primarily through public offerings of our common stock, sales of our common stock pursuant to our at-the-market equity offering programs, our loan and security agreement with Hercules Capital Inc. (Hercules), and the issuance of our 5.00% Convertible Senior Notes due 2048 (the Notes). As of December 31, 2018, there was \$25.0 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:

- commercialize COPIKTRA;





## Table of Contents

- continue our ongoing clinical trials with our product candidates, including with COPIKTRA 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 commercialization efforts; and
- establish and maintain a sales, marketing and distribution infrastructure to commercialize COPIKTRA or 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, such as COPIKTRA. 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 COPIKTRA.

We expect our expenses to increase in connection with our ongoing activities, particularly as we commercialize COPIKTRA and continue the clinical development of our other product candidates. We expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution of COPIKTRA. 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 COPIKTRA.

We expect our cash, cash equivalents and investments at December 31, 2018 will be sufficient to fund our current operating plan and capital expenditure requirements beyond the next twelve months. Our future capital requirements will depend on many factors, including:

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

Conducting clinical trials is a time consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval of any of our other product candidates. Even though the FDA approved COPIKTRA, it may not achieve commercial success. Our commercial revenues will be derived from sales of products, such as COPIKTRA. Accordingly, even though we received

regulatory approval for COPIKTRA, 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.

## Table of Contents

### 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, March and October 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. As of December 31, 2018, there was \$25.0 million available to borrow under the Amended Loan Agreement, subject to certain conditions of financing. All obligations under the Amended Loan Agreement are secured by substantially all of our existing property and assets, excluding our intellectual property.

In October 2018, we closed a registered direct public offering of \$150.0 million aggregate principal amount of the Notes. The Notes are governed by the terms of a base indenture for senior debt securities (the Base Indenture), as supplemented by the first supplemental indenture thereto (the Supplemental Indenture and together with the Base Indenture, the Indenture). The Notes are senior unsecured obligations of the Company and bear interest at a rate of 5.00% per annum, payable semi-annually in arrears on May 1 and November 1 of each year, beginning on May 1, 2019. The Notes will mature on November 1, 2048, unless earlier repurchased, redeemed or converted in accordance with their terms.

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 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 the Indenture, or breaching any covenants under the Amended Loan Agreement or the Indenture, subject to specified cure periods with respect to certain breaches, could result in an event of default and, as a result, could accelerate all of the amounts due. Further, the Notes are subject to repurchase by us, at the option of the holders, at certain dates as specified within the Indenture prior to maturity in 2048. In the event of an acceleration of amounts due under the Amended Loan Agreement or the Indenture, 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 COPIKTRA commercialization efforts, other product candidate development or grant to others the rights to develop and market COPIKTRA and our other 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.



## Table of Contents

The Amended Loan Agreement and the Indenture impose 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 commercialize COPIKTRA or obtain regulatory approvals for and commercialize any of our other product candidates.

We rely on third parties, such as 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 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 some of our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize COPIKTRA and our other product candidates.



## Table of Contents

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, including COPIKTRA, 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 COPIKTRA and our other 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 quantities of our product candidates and commercial quantities of COPIKTRA. In addition, we currently rely on third parties for the development of various formulations of COPIKTRA and our other product candidates. This reliance on third parties increases the risk that we will not have sufficient quantities of COPIKTRA or our product candidates or such quantities at an acceptable cost or quality, which could delay, prevent or impair our development or commercialization efforts.

We do not currently have arrangements in place for redundant supply or a second source for bulk drug substance or drug product. Even though we have supply agreements in place with our third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

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



manufacturers or suppliers.

55

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## Table of Contents

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. Any interruption of the development or operation of the manufacturing facilities due to, among other reasons, events such as order delays for equipment or materials, equipment malfunction, quality control and quality assurance issues, regulatory delays and possible negative effects of such delays on supply chains and expected timelines for product availability, production yield issues, shortages of qualified personnel, discontinuation of a facility or business or failure or damage to a facility by natural disasters, could result in the cancellation of shipments, loss of product in the manufacturing process or a shortfall in available COPIKTRA, other product candidates or materials.

If our current contract manufacturers cannot perform as agreed or these parties cease to provide quality manufacturing and related services to us, we may be required to replace that manufacturer. If we are not able to engage appropriate replacements in a timely manner, our ability to manufacture COPIKTRA or our other product candidates in sufficient quality and quantity required for commercial use of COPIKTRA and/or for planned pre-clinical testing, clinical trials and potential commercial use of our product candidates would be adversely affected. 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 and obtaining regulatory approvals for the new manufacturer. 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. In light of the lead time needed to manufacture COPIKTRA and our other product candidates, and the availability of underlying materials, we may not be able to, in a timely manner or at all, establish or maintain sufficient commercial manufacturing arrangements on commercially reasonable terms necessary to provide adequate supply of COPIKTRA to meet demands that exceed our commercial assumptions or to provide adequate supply of our other product candidates to meet demands that exceed our clinical assumptions. Furthermore, we may not be able to obtain the significant financial capital that may be required in connection with such arrangements. Even after successfully engaging third parties to execute the manufacturing process for COPIKTRA and our other product candidates, such parties may not comply with the terms and timelines they have agreed to for various reasons, some of which may be out of their or our control, which could impact our ability to execute our business plans on expected or required timelines in connection with the commercialization of COPIKTRA and the continued development of our other product candidates. We may also be required to enter into long-term manufacturing agreements that contain exclusivity provisions and/or substantial termination penalties, which could have a material adverse effect on our business prior to and after commercialization.

Our current and anticipated future dependence upon others for the manufacture of our other 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

56

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## Table of Contents

FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate. Collaborations are complex and time consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

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

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

We may seek third-party collaborators for the development and commercialization of our product candidates. For instance, we have entered into agreements for the development and commercialization of COPIKTRA in China, Hong Kong, Macau and Taiwan with CSPC Pharmaceutical Group Limited and in Japan with Yakult Honsha Co., Ltd. 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;

## Table of Contents

- disputes may arise between the collaborators and us that result in the delay or termination of the research, development or commercialization of our products or product candidates or that result in costly litigation or arbitration that diverts management attention and resources; and
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.

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

If we are unable to maintain our agreements with third parties to distribute COPIKTRA to patients, our results of operations and business could be adversely affected.

We will continue to rely on third parties to commercially distribute COPIKTRA to patients. We have contracted with a third-party logistics company to warehouse COPIKTRA and to process and ship customer orders, and with specialty pharmacies and specialty distributors to sell and distribute COPIKTRA. The specialty pharmacies sell COPIKTRA directly to patients and provide patient education and ongoing management. The specialty distributors sell COPIKTRA to community oncologists with in-office dispensaries, hospital-owned practices, local offices with onsite pharmacies, retail pharmacies, and other institutional customers. We have also contracted with a third-party patient services hub to help us with some or all of the following: reimbursement adjudication, patient financial support, patient assistance programs and ongoing compliance support. This distribution network will require significant coordination with our sales and marketing and finance organizations. In addition, failure to coordinate financial systems could negatively impact our ability to accurately report product revenue from COPIKTRA. If we are unable to effectively manage the distribution process, the commercial launch and sales of COPIKTRA, as well as any future products we may commercialize, sales could be delayed or severely compromised and our results of operations may be harmed.

In addition, the use of specialty pharmacies, specialty distributors and a call center involve certain risks, including, but not limited to, risks that these organizations will:

- not provide us with accurate or timely information regarding their inventories, the number of patients who are using COPIKTRA or serious adverse reactions, events and/or product complaints regarding COPIKTRA;
- not effectively sell or support COPIKTRA, or communicate publicly concerning COPIKTRA in a manner that is contrary to FDA rules and regulations;
- reduce or discontinue their efforts to sell or support COPIKTRA;
- not devote the resources necessary to sell COPIKTRA in the volumes and within the time frame we expect;
- be unable to satisfy financial obligations to us or others; or
- cease operations.

Any such events may result in decreased product sales and lower product revenue, which would harm our results of operations and business.

## 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 Inc., or 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,

58

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## Table of Contents

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 COPIKTRA or 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 COPIKTRA.

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.

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, 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-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,

## Table of Contents

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 commercialize, develop, manufacture, market and sell COPIKTRA and our other 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 COPIKTRA and our other 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.



## Table of Contents

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

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

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

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development, 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.

## Risks Related to Maintaining and Expanding COPIKTRA's Regulatory Approval, Achieving Regulatory Approval of Our Other 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 our product candidates, we will not be able to commercialize such candidates, and our ability to generate revenue will be

materially impaired.

Obtaining approval of an NDA can be a lengthy, expensive and uncertain process. The activities associated with a product candidate's development and commercialization, including its design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution are subject to

61

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## Table of Contents

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 product candidates will prevent us from commercializing such product candidates. We have not received approval to market any of our product candidates from regulatory authorities in any jurisdiction, except for COPIKTRA in the United States. 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. A product candidate 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 a product candidate, its commercial prospects may be harmed and our ability to generate revenues will be materially impaired.

We have received orphan drug designation for COPIKTRA and 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 COPIKTRA or these product candidates.

We received orphan drug designation in the United States and the European Union for the use of COPIKTRA in CLL/SLL and FL, in the United States and European Union for the use of defactinib in ovarian cancer, and in the United States, the European Union, and Australia for the use of defactinib in mesothelioma. 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. Other companies have received orphan drug designations for compounds other than COPIKTRA or defactinib for the same indications for which we may have received orphan drug designation in corresponding territories. While orphan drug exclusivity for COPIKTRA or defactinib provides 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 orphan designation criteria are no longer met or if the drug is sufficiently profitable so that market exclusivity is no longer justified. 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.

62

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## Table of Contents

We may seek fast track designation for COPIKTRA in additional indications, or for one or more of our other product candidates, but we might not receive such designation, and even if we do, such 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 for COPIKTRA for the treatment of patients with peripheral T-cell lymphoma who have received at least one prior therapy. Any sponsor may seek fast track designation for a drug if it is intended for the treatment of a serious condition and nonclinical or clinical data demonstrate the potential to address unmet medical need for this condition, a drug sponsor may apply for FDA fast track designation. If we seek fast track designation for a product candidate, we may not receive it from the FDA. However, even if we receive fast track designation, fast track designation does not ensure that we will receive marketing approval or that approval will be granted within any particular timeframe. We may not experience a faster development or regulatory review or approval process with fast track designation compared to conventional FDA procedures. In addition, the FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program. Fast track designation alone does not guarantee qualification for the FDA's priority review procedures.

COPIKTRA and any other 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.

COPIKTRA and any other product candidate for which we obtain marketing approval, along with the manufacturing processes, post approval clinical data, labeling, advertising and promotional activities for such product, will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post marketing information and reports, registration and listing requirements, cGMP requirements relating to quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for costly post marketing testing and surveillance to monitor the safety or efficacy of the product, including the imposition of a REMS.

With respect to COPIKTRA, the indication in FL is approved by the FDA under accelerated approval based on overall response rate observed in clinical trials. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials. The FDA is requiring that we conduct a clinical trial in patients with relapsed or refractory FL that verifies and isolates the benefits of COPIKTRA. Additionally, as a requirement of the FDA's approval, we are implementing an informational REMS that entails a communication plan to provide appropriate dosing and safety information to better support physicians in managing their patients on COPIKTRA.

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;

## Table of Contents

- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenue;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our products;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

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 earnings.

Healthcare providers, including physicians, and third-party payors play a primary role in the recommendation and prescription of COPIKTRA and any other product candidates for which we obtain marketing approval. Our arrangements with healthcare providers, third-party payors and other parties within the healthcare industry 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 COPIKTRA and any products for which we obtain marketing approval. Restrictions under applicable federal and state healthcare and regulatory laws and regulations within the United States 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), which 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 civil monetary penalties laws, which impose civil fines for, among other things, the offering or transfer of remuneration to a Medicare or state healthcare program beneficiary if the person knows or should know it is likely to influence the beneficiary's selection of a particular provider, practitioner, or supplier of services reimbursable by Medicare or a state healthcare program, unless an exception applies;
- 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;



Table of Contents

- the federal anti-kickback prohibition known as Eliminating Kickbacks in Recovery Act or EKRA, enacted in 2018, which prohibits certain payments related to referrals of patients to certain providers (recovery homes, clinical treatment facilities and laboratories) and applies to services reimbursed by private health plans as well as government health care programs;
- 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;
- federal and state consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers;
- the so-called federal "sunshine law" or Open Payments that 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 hospital and, beginning with transfers of value occurring in 2021, other healthcare practitioners, as well as ownership and investment interests held by physicians and their immediate family members; and
- analogous state laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non- governmental third-party payors, including private insurers, and some state laws 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.

Similar healthcare laws and regulations exist in the European Union and other foreign jurisdictions, including reporting requirements detailing interactions with and payments to healthcare providers and laws governing the privacy and security of certain protected information. For example, in May 2018, a new privacy regime, the General Data Protection Regulation (GDPR), took effect enhancing our obligations with respect to operations in the European Economic Area, or the EEA, and increasing the scrutiny applied to transfers of personal data from the EEA (including health data from our clinical sites in the EEA) to countries that are considered by the European Commission to lack an adequate level of data protection, such as the United States. The compliance obligations imposed by the GDPR have required us to revise our operations and increased our cost of doing business. In addition, the GDPR imposes substantial fines for breaches of data protection requirements, and it confers a private right of action on data subjects for breaches of data protection requirements.

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, or patient assistance programs, 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, additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, 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 is 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. Further, defending against any such actions can be costly, time-consuming and may require significant personnel resources. Therefore, even if we are successful in defending

against any such actions that may be brought against us, our business may be impaired.

65

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## Table of Contents

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 commercialize COPIKTRA, obtain marketing approval of and commercialize our other 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 COPIKTRA and any other 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 extended 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.

The provisions of the Healthcare Reform Act have been subject to judicial and Congressional challenges, as well as efforts by the Trump administration to modify certain requirements of the Healthcare Reform Act by executive branch order. For example, on January 20, 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under the Healthcare Reform Act to waive, defer, grant exemptions from, or delay the implementation of any provision of the Healthcare Reform Act that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. On October 12, 2017, President Trump signed another Executive Order directing certain federal agencies to propose regulations or guidelines to provide small businesses with greater opportunities to form association health plans, expand the availability of short-term, limited duration insurance, and allow employees to make use of certain employer-paid health benefits, called health reimbursement arrangements, to pay for health insurance that does not meet all Healthcare Reform Act requirements. In addition, citing legal guidance from the U.S. Department of Justice,

the U.S. Department of Health and Human Services, or HHS, concluded that cost-sharing reduction, or CSR, payments to insurance companies required under the Healthcare Reform Act had not received necessary appropriations from Congress. President Trump subsequently discontinued

## Table of Contents

these payments. The loss of the CSR payments is expected to increase premiums on certain policies issued by qualified health plans under the Healthcare Reform Act. Certain administrative actions have been subject to judicial challenge. In Congress, there have been a number of legislative initiatives to modify, repeal and/or replace portions of the Healthcare Reform Act. Tax reform legislation enacted at the end of 2017 eliminated the tax penalty for individuals who do not maintain sufficient health insurance coverage beginning in 2019. The Bipartisan Budget Act of 2018 contained various provisions that affect coverage and reimbursement of drugs, including an increase in the discount that manufacturers of Medicare Part D brand name drugs must provide to Medicare Part D beneficiaries during the coverage gap from 50% to 70% starting in 2019. Congress may consider other legislation to modify, repeal and/or replace certain elements of the Healthcare Reform Act. In December 2018, a federal district court judge, in a challenge brought by a number of state attorneys general, found the Healthcare Reform Act unconstitutional in its entirety because, once Congress repealed the individual mandate provision, there was no longer a basis to rely on Congressional taxing authority to support enactment of the law. Pending appeals, which could take some time, the Healthcare Reform Act is still operational in all respects. We continue to evaluate the effect that the Healthcare Reform Act and its possible repeal, replacement or modification may have on our business. Such legislation and other healthcare reform measures that may be adopted in the future could have a material adverse effect on our industry generally and on our ability to successfully commercialize our products and product candidates.

In addition, other broader legislative changes have been adopted that could have an adverse effect upon, and could prevent, our products' commercial success. The Budget Control Act of 2011, as amended, or the Budget Control Act, includes provisions intended to reduce the federal deficit, including reductions in Medicare payments to providers through 2027. Any significant spending reductions affecting Medicare, Medicaid or other publicly funded or subsidized health programs, or any significant taxes or fees imposed as part of any broader deficit reduction effort or legislative replacement to the Budget Control Act, or otherwise, could have an adverse impact on our anticipated product revenues.

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 constraints, restrictions on copayment assistance by pharmaceutical manufacturers, marketing cost disclosure and transparency measures, and, in some cases, measures designed to encourage importation from other countries and bulk purchasing.

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 COPIKTRA or the marketing approvals of our product candidates 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, Robert Gagnon, our Chief Financial 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, Robert Gagnon 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

## Table of Contents

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.

We may expand our development, regulatory and 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 COPIKTRA and our other 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;

## Table of Contents

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

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

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

Our stock price has been volatile. Since January 27, 2012, when we became a public company, the price for one share of our common stock has reached a high of \$18.82 and a low of \$1.05 through December 31, 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; the success of commercializing COPIKTRA;
- 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.



## Table of Contents

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 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.

We have limited experience in marketing and commercializing product candidates. If we are unable to successfully maintain and further develop internal commercialization capabilities, establish sales and marketing capabilities or enter into agreements with third parties to sell and market our product candidates, sales of COPIKTRA may be negatively impacted and we may not be successful in commercializing our other product candidates if and when they are approved.

We 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.

We have hired a commercial team and implemented the organizational infrastructure we believe we need for a successful commercial launch of COPIKTRA. We will need to commit significant time and financial and managerial resources to maintain and further develop our marketing and sales force to ensure they have the technical expertise required to address any challenges we may face with the commercialization of COPIKTRA. Factors that may inhibit our efforts to maintain and develop our commercialization capabilities include:

- an inability to retain an adequate number of effective commercial personnel;
- an inability to train sales personnel, who may have limited experience with our company or COPIKTRA, to deliver a consistent message regarding COPIKTRA and be effective in persuading physicians to prescribe COPIKTRA;
- an inability of sales personnel to obtain access to physicians or persuade adequate numbers of physicians to prescribe COPIKTRA or any other product candidates;
- an inability of third-parties to manufacture COPIKTRA consistently in commercial quantities, at acceptable costs and on expected timelines;
- a 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;
- an inability to equip sales personnel with effective materials, including medical and sales literature to help them educate physicians and other healthcare providers regarding COPIKTRA; and
- unforeseen costs and expenses associated with maintaining and further developing an independent sales and marketing organization.

If we are not successful in establishing and maintaining an effective sales and marketing infrastructure, we will have difficulty commercializing COPIKTRA, which would adversely affect our business and financial condition.

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



Table of Contents

Risks Related to the Notes

Servicing our debt, including the Notes, requires a significant amount of cash, and we may not have sufficient cash flow from our business to pay our substantial debt.

Our ability to make scheduled payments of the principal of, to pay interest on or to refinance our indebtedness, including the Notes, depends on the timing of regulatory reviews and approvals and our future performance, which is subject to regulatory, economic, financial, competitive and other factors beyond our control. We are a clinical stage biopharmaceutical company and we have not yet generated any profit from product sales. We expect to continue to incur losses as we continue our clinical development of, and seek regulatory approvals for, our product candidates, prepare to commercialize any approved products and add infrastructure and personnel to support our product development efforts and operations. Accordingly, our business may not generate cash flow from operations in the future sufficient to service our debt and make necessary capital expenditures. If we are unable to generate such cash flow, we may be required to adopt one or more alternatives, such as selling assets, restructuring debt or obtaining additional equity capital on terms that may be onerous or highly dilutive. Our ability to refinance our indebtedness will depend on the capital markets and our financial condition at such time. We may not be able to engage in any of these activities or engage in these activities on desirable terms, which could result in a default on our debt obligations.

The Notes are effectively subordinated to our secured indebtedness and structurally subordinated to any liabilities of our subsidiaries.

The Notes are our senior, unsecured obligations and are senior in right of payment to our future indebtedness that is expressly subordinated in right of payment to the Notes; equal in right of payment with our existing and future indebtedness that is not so subordinated, and effectively subordinated to our existing and future secured indebtedness, to the extent of the value of the collateral securing such indebtedness. The Notes are structurally subordinated to all existing and future indebtedness and other liabilities, including trade payables, and (to the extent we are not a holder thereof) preferred equity, if any, of our subsidiaries. In the event of our bankruptcy, liquidation, reorganization or other winding up, our assets that secure debt will be available to pay obligations on the Notes only after the secured debt has been repaid in full from these assets, and the assets of our subsidiaries will be available to pay obligations on the Notes only after all claims of such subsidiaries' creditors, including trade creditors and preferred equity holders have been repaid in full. There may not be sufficient assets remaining to pay amounts due on any or all of the Notes then outstanding. The indenture and supplemental indenture governing the Notes do not prohibit us from incurring additional senior debt or secured debt, nor do they prohibit any of our subsidiaries from incurring additional liabilities.

Despite our current debt levels, we may still incur substantially more debt or take other actions which would intensify the risks discussed above.

Despite our current consolidated debt levels, we and our subsidiaries may be able to incur substantial additional debt in the future, subject to the restrictions contained in our debt agreements, some of which may be secured debt. We are not restricted under the terms of the indenture or the supplemental indenture governing the Notes from incurring additional debt, securing existing or future debt, recapitalizing our debt or taking a number of other actions that are not limited by the terms of the indenture or the supplemental indenture governing the Notes that could have the effect of diminishing our ability to make payments on the Notes when due. While the Amended Loan Agreement, as amended by the Third Amendment, restricts our ability and the ability of our subsidiaries to issue or incur additional indebtedness, including secured indebtedness, if our loans under the Amended Loan Agreement, as amended by the Third Amendment, mature or are repaid, we may not be subject to such restrictions under the terms of any subsequent indebtedness.





## Table of Contents

We may not have the ability to raise the funds necessary to repurchase the Notes upon a fundamental change, and our existing or future debt may contain limitations on our ability to repurchase the Notes.

Holders of the Notes have the right to require us to repurchase their Notes upon the occurrence of a fundamental change at a fundamental change repurchase price equal to 100% of the principal amount of the Notes to be repurchased, plus accrued and unpaid interest, if any. However, we may not have enough available cash or be able to obtain financing at the time we are required to make repurchases of Notes surrendered therefor. In addition, our ability to repurchase the Notes may be limited by law, by regulatory authority or by agreements governing our indebtedness that exist at the time of the repurchase. The Amended Loan Agreement, as amended by the Third Amendment, currently limits our ability to repurchase the Notes. Our failure to repurchase Notes at a time when the repurchase is required by the indenture and supplemental indenture governing the Notes would constitute a default under the indenture and supplemental indenture. A default under the indenture or the supplemental indenture or the fundamental change itself could also lead to a default under the Amended Loan Agreement, as amended by the Third Amendment, and/or agreements governing our future indebtedness. If the repayment of the related indebtedness were to be accelerated after any applicable notice or grace periods, we may not have sufficient funds to repay the indebtedness and repurchase the Notes.

In addition, our borrowings under the Amended Loan Agreement, as amended by the Third Amendment, are, and are expected to continue to be, at variable rates of interest and expose us to interest rate risk. If interest rates increase, our debt service obligations on the variable rate indebtedness would increase even though the amount borrowed remained the same, and our net income would decrease.

The Amended Loan Agreement, as amended by the Third Amendment, limits our ability to pay any cash amount upon repurchase of the Notes.

The Amended Loan Agreement, as amended by the Third Amendment, prohibits us from making any cash payments to repurchase the Notes upon a fundamental change. Any new credit facility that we may enter into may have similar restrictions.

Our failure to repurchase the Notes as required under the terms of the Notes would constitute a default under the indenture and the supplemental indenture governing the Notes and would permit holders of the Notes to accelerate our obligations under the Notes. A default under the indenture or the supplemental indenture or the fundamental change itself could also lead to a default under the Amended Loan Agreement, as amended by the Third Amendment, or agreements governing our future indebtedness. If the repayment of the related indebtedness were to be accelerated after any applicable notice or grace periods, we may not have sufficient funds to repay the indebtedness and repurchase the Notes.

Future sales of our common stock or equity-linked securities in the public market could lower the market price for our common stock.

In the future, we may sell additional shares of our common stock or equity-linked securities to raise capital. In addition, a substantial number of shares of our common stock are reserved for issuance upon the exercise of stock options and upon conversion of the Notes. We cannot predict the size of future issuances or the effect, if any, that they may have on the market price for our common stock. The issuance and sale of substantial amounts of common stock or equity-linked securities, or the perception that such issuances and sales may occur, could adversely affect the market price of our common stock and impair our ability to raise capital through the sale of additional equity or equity-linked securities.

Item 1B. Unresolved Staff Comments

None.

72

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## Table of Contents

### Item 2. Properties

We occupy approximately 27,810 square feet of office space in Needham, Massachusetts under a lease that expires in May 2025. 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

From time to time, we are subject to various legal proceedings and claims that arise in the ordinary course of our business activities. We do not believe we are currently party to any pending legal action, the outcome of which, if determined adversely to us, would individually or in the aggregate be reasonably expected to have a material adverse effect on our business or operating results.

### 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.”

#### HOLDERS

As of February 28, 2019, there were 15 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 \$3.01. 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.

#### 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, 2013 through December 31, 2018. The comparison assumes \$100 was invested after the market closed on December 31, 2013 in our common stock and in each of the foregoing indices, and it assumes reinvestment of dividends, if any.



Table of Contents

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 12/31/13 in stock or index, including reinvestment of dividends. Fiscal year ending December 31, 2018.

Cumulative Total Return Comparison

	December 31,					
	2013	2014	2015	2016	2017	2018
Verastem, Inc.	100.00	80.18	16.32	9.82	26.93	29.47
NASDAQ Composite	100.00	114.62	122.81	133.19	172.11	165.84
NASDAQ Biotechnology	100.00	131.71	140.56	112.25	133.67	121.24

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.

Table of Contents

## 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,				
	2018	2017	2016	2015	2014
	(in thousands, except share and per share amounts)				
Revenue:					
Product revenue, net	\$ 1,718	—	—	—	—
License revenue	25,000	—	—	—	—
Total revenue	26,718	—	—	—	—
Operating expenses:					
Costs of revenues, excluding amortization of acquired intangible assets	\$ 165	\$ —	\$ —	\$ —	\$ —
Research and development	43,648	46,423	19,779	40,565	35,448
Selling, general and administrative	77,265	21,381	17,223	17,634	18,159
Amortization of acquired intangible assets	423	—	—	—	—
Total operating expenses	121,501	67,804	37,002	58,199	53,607
Loss from operations	(94,783)	(67,804)	(37,002)	(58,199)	(53,607)
Other income	25,556	—	—	—	—
Interest income	2,603	561	562	334	242
Interest expense	(5,810)	(559)	—	—	—
Net loss applicable to common stockholders	\$ (72,434)	\$ (67,802)	\$ (36,440)	\$ (57,865)	\$ (53,365)
Net loss per share applicable to common stockholders—basic	\$ (1.12)	\$ (1.76)	\$ (0.99)	\$ (1.61)	\$ (2.07)
Net loss per share applicable to common stockholders—diluted	\$ (1.37)	\$ (1.76)	\$ (0.99)	\$ (1.61)	\$ (2.07)
Weighted average common shares outstanding used in computing:					
Net loss per share applicable to common stockholders—basic	64,962	38,422	36,988	35,932	25,804
Net loss per share applicable to common stockholders—diluted	69,321	38,422	36,988	35,932	25,804
Balance sheet data:	As of December 31,				
	2018	2017	2016	2015	2014
	(in thousands)				
	\$ 249,653	\$ 86,672	\$ 80,897	\$ 110,258	\$ 92,675

Cash, cash equivalents and investments					
Working capital	216,182	70,659	70,304	100,734	86,112
Total assets	277,236	89,791	83,629	113,094	98,649
Accumulated deficit	(375,576)	(303,142)	(235,323)	(198,883)	(141,018)
Total stockholders' equity	124,299	57,684	72,297	102,469	88,766



## Table of Contents

### 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 medicines to improve the survival and quality of life of cancer patients. Both our marketed product, COPIKTRA™ (duvelisib) capsules, and most advanced product candidate, defactinib, utilize a multi-faceted approach to treat cancers originating either in the blood or major organ systems. We are currently developing our product candidates in both preclinical and clinical studies as potential therapies for certain cancers, including leukemia, lymphoma, lung cancer, ovarian cancer, mesothelioma, and pancreatic cancer.

Our operations to date have been organizing and staffing our company, business planning, raising capital, identifying and acquiring potential product candidates, undertaking preclinical studies and clinical trials for our product candidates and scaling up U.S. commercial operations in anticipation for our first approved drug candidate. We have financed our operations to date primarily through public offerings of our common stock, sales of common stock under our at-the-market equity offering programs, our loan and security agreement executed with Hercules Capital, Inc. (Hercules) in March 2017, as amended, the upfront payments under our license and collaboration agreements with Yakult and CSPC, and the issuance in October 2018 of \$150.0 million aggregate principal amount of Notes. With our U.S. commercial launch of COPIKTRA on September 24, 2018, we have recently begun financing a portion of our operations through product revenue.

As of December 31, 2018, we had an accumulated deficit of \$375.6 million. Our net loss was \$72.4 million, \$67.8 million and \$36.4 million for the years ended December 31, 2018, 2017 and 2016, respectively. We expect to incur significant expenses and increasing operating losses for the foreseeable future as a result of our commercialization of COPIKTRA and the continued research and development of all of our product candidates. We will need to generate significant revenues to achieve profitability, and we may never do so. As of December 31, 2018, we had cash, cash equivalents and short-term investments of \$249.7 million. We expect, prior to the consideration of any revenue from future potential sales of COPIKTRA, that our cash, cash equivalents and short-term investments will be sufficient to fund our U.S. commercial scale-up, development plans and other operational activities for at least twelve months from the date of issuance of our consolidated financial statements.

We expect to finance the future development costs of our clinical product portfolio with our existing cash, cash equivalents and short-term investments, or through strategic financing opportunities that could include, but are not limited to collaboration agreements, future offerings of our equity, or the incurrence of debt. However, there is no guarantee that any of these strategic or financing opportunities will be executed or executed on favorable terms, and some could be dilutive to existing stockholders. If we fail to obtain additional future capital, we may be unable to complete our planned preclinical studies and clinical trials and obtain approval of certain investigational product candidates from the FDA or foreign regulatory authorities.

#### FINANCIAL OPERATIONS OVERVIEW

Revenue

Product revenue, net represents the gross sales of COPIKTRA in the United States less provisions for product sales allowances and accruals. These provisions include trade allowances, rebates, chargebacks and discounts, product returns and other incentives. We sell COPIKTRA to a limited number of specialty pharmacies and specialty distributors. Although we expect net product revenues to increase over time, the provisions for product sales and allowances may fluctuate based on the mix of sales to either specialty pharmacy or specialty

76

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## Table of Contents

distributor customers. See “Critical Accounting Policies and Significant Judgements and Estimates” below for more information on the components of net U.S. product sales of COPIKTRA.

License revenue to date has been generated through our license and collaboration agreements for the development and commercialization of duvelisib with CSPC in China and Yakult in Japan. The terms of these agreements contain multiple deliverables which may include (i) licenses, (ii) research and development activities, and (iii) the manufacture of finished drug product, active pharmaceutical ingredient (API), or development materials for a partner, which are reimbursed at a contractually determined rate. Payments to us may include (i) up front license fees, (ii) payments for research and development activities, (iii) payments for the manufacture of finished drug product, API or development materials, (iv) payments based upon the achievement of certain milestones, and (v) royalties on product sales. Duvelisib has not received regulatory approval for commercial sale in either China or Japan.

### Costs of revenues, excluding amortization of acquired intangibles

Costs of revenues, excluding amortization of acquired intangibles consists of the costs of COPIKTRA on which product revenue was recognized and royalties we incur as a result of sales of COPIKTRA. Our costs of revenue initially consists of capsule production, packaging, and product shipment. During the third quarter of 2018, we began capitalizing inventory costs of COPIKTRA based on our evaluation of the ability of our third-party suppliers to successfully manufacture commercial quantities of COPIKTRA and the likelihood of approval of the New Drug Application (NDA) in the United States. Any production costs for COPIKTRA prior to this time, which included the costs to manufacture drug product, in addition to the costs noted above, were included in research and development costs. To date, any API and raw starting materials used in the manufacturing of COPIKTRA was inherited pursuant to the license agreement executed with Infinity and, as such, the Company has not recorded any inventory or expenses related to API or raw starting materials. We expect costs of revenue to increase as net product revenues increase and as a result of increased capitalized costs, which will include the cost of API and drug substance, associated with the production of COPIKTRA in future periods.

### 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;
- 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; and
- costs associated with COPIKTRA prior to us concluding that regulatory approval is probable and that its net realizable value is recoverable.

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.



Table of Contents

On September 24, 2018, COPIKTRA was approved by the FDA and is now indicated for the treatment of adult patients with relapsed or refractory CLL/SLL after at least two prior therapies and relapsed or refractory FL after at least two prior systemic therapies. Due to long-lead time requirements for manufacturing our product, manufacturing constraints and the desire to have COPIKTRA commercially available as soon as possible following regulatory approval, we contracted with our third-party supplier to manufacture commercial quantities of COPIKTRA drug substance prior to final approval by regulators.

To date, any API and raw starting materials used in the manufacturing of COPIKTRA was inherited pursuant to the license agreement executed with Infinity and, as such, we have not recorded any inventory or expenses related to API or raw starting materials. We expensed all pre-validation and validation manufacturing costs of drug product as research and development expenses in the periods prior to July 1, 2018. Total costs of manufacturing COPIKTRA drug product expensed as research and development through June 30, 2018 was approximately \$1.8 million. Beginning July 1, 2018, we began capitalizing COPIKTRA related drug product costs for validation and post-validation (i.e. commercial) lots as regulatory approval became probable. For the periods beginning on July 1, 2018 and beyond, we have capitalized any COPIKTRA drug product costs incurred for commercial use as inventory.

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 COPIKTRA and defactinib, for the years ended December 31, 2018, 2017 and 2016. 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 \$9.2 million, \$5.8 million and \$3.9 million of personnel costs for the years ended December 31, 2018, 2017 and 2016, respectively.

	Year ended December 31,		
	2018 (in thousands)	2017 (in thousands)	2016 (in thousands)
COPIKTRA	\$ 24,771	\$ 30,409	\$ 3,326
Defactinib	2,230	2,894	3,934
Unallocated and other research and development expense	14,604	11,739	11,445
Unallocated stock-based compensation expense	2,043	1,381	1,074
Total research and development expense	\$ 43,648	\$ 46,423	\$ 19,779

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 COPIKTRA or any of our other 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.

## Table of Contents

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.

### Selling, general and administrative expenses

Selling, general and administrative expenses consist primarily of salaries and related costs for personnel, including stock based compensation expense, in our executive, finance, commercial and business development functions. Other selling, general and administrative expenses include allocated facility costs, commercial supply costs not capitalized as inventory, professional fees for legal, patent, investor and public relations, consulting, insurance premiums, audit, tax and other public company costs.

### Other, interest income and interest expense

Other income consists entirely of the mark-to-market adjustment of the bifurcated conversion option derivative liability related to the Notes.

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

Interest expense reflects interest expense due under both our term loan facility executed with Hercules and the Notes, as well as 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, revenues 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, stock based compensation, revenue recognition, collaborative agreements, accounts receivable, inventory and intangible assets 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.

Table of Contents

## Revenue Recognition

Effective January 1, 2018, we adopted Accounting Standards Codification (ASC) 606 Revenue from Contracts with Customers. This standard applies to all contracts with customers, except for contracts that are within the scope of other standards, such as leases, insurance, collaboration arrangements and financial instruments. Under ASC 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements that an entity determines are within the scope of ASC 606, the entity performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation. We only apply the five-step model to contracts when it is probable that we will collect the consideration we are entitled to in exchange for the goods or services we transfer to the customer. At contract inception, once the contract is determined to be within the scope of ASC 606, we assess the goods or services promised within each contract and determine which goods or services are performance obligations, and assess whether each promised good or service is distinct. We then recognize as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

Product Revenue, Net – We sell COPIKTRA to a limited number of specialty pharmacies and specialty distributors in the United States. These customers subsequently resell COPIKTRA either directly to patients, or to community hospitals or oncology clinics with in-office dispensaries who in turn distribute COPIKTRA to patients. In addition to distribution agreements with customers, we also enter into arrangements with (1) certain government agencies and various private organizations (Third-Party Payers), which may provide for chargebacks or discounts with respect to the purchase of COPIKTRA, and (2) Medicare and Medicaid, which may provide for certain rebates with respect to the purchase of COPIKTRA.

We recognize revenue on sales of COPIKTRA when a customer obtains control of the product, which occurs at a point in time (typically upon delivery). Product revenues are recorded at the wholesale acquisition costs, net of applicable reserves for variable consideration. Components of variable consideration include trade discounts and allowances, Third-Party Payer chargebacks and discounts, government rebates, other incentives, such as voluntary co-pay assistance, product returns, and other allowances that are offered within contracts between us and customers, payors, and other indirect customers relating to our sale of COPIKTRA. These reserves, as detailed below, are based on the amounts earned, or to be claimed on the related sales, and are classified as reductions of accounts receivable or a current liability. These estimates take into consideration a range of possible outcomes based upon relevant factors such as, customer contract terms, information received from third-parties regarding the anticipated payor mix for COPIKTRA, known market events and trends, industry data, and forecasted customer buying and payment patterns. Overall, these reserves reflect our best estimates of the amount of consideration to which we are entitled with respect to sale made.

The amount of variable consideration which is included in the transaction price may be constrained and is included in the net sales price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized under contracts will not occur in a future period. Our analyses contemplate the application of the constraint in accordance with ASC 606. For the year ended December 31, 2018, we determined a material reversal of revenue would not occur in a future period for the estimates detailed below and, therefore, the transaction price was not reduced further. Actual amounts of consideration ultimately received may differ from our estimates. If actual results in the future vary from our estimates, we will adjust these estimates, which would affect net product revenue and earnings in the period such variances become known.



Trade Discounts and Allowances: We generally provide customers with invoice discounts on sales of COPIKTRA for prompt payment, which are explicitly stated in our contracts and are recorded as a reduction of revenue in the period the related product revenue is recognized. In addition, we compensate our specialty distributor customers for sales order management, data, and distribution services. We have determined such services are not distinct from our sale of COPIKTRA to the specialty distributor customers and, therefore, these payments have also been recorded as a reduction of revenue within the consolidated statements of operations and comprehensive loss through December 31, 2018.

## Table of Contents

**Third-Party Payer Chargebacks, Discounts and Fees:** We execute contracts with Third-Party Payers which allow for eligible purchases of COPIKTRA at prices lower than the wholesale acquisition cost charged to customers who directly purchase the product from us. In some cases, customers charge us for the difference between what they pay for COPIKTRA and the ultimate selling price to the Third-Party Payers. These reserves are established in the same period that the related revenue is recognized, resulting in a reduction of product revenue and accounts receivable, net. Chargeback amounts are generally determined at the time of resale to the qualified Third-Party Payer by customers, and we generally issue credits for such amounts within a few weeks of the customer's notification to us of the resale. Reserves for chargebacks consist of credits that we expect to issue for units that remain in the distribution channel inventories at the end of each reporting period that we expect will be sold to Third-Party Payers, and chargebacks that customers have claimed, but for which we have not yet issued a credit. In addition, we compensate certain Third-Party Payers for administrative services, such as account management and data reporting. These administrative service fees have also been recorded as a reduction of product revenue within the consolidated statements of operations and comprehensive loss through December 31, 2018.

**Government Rebates:** We are subject to discount obligations under state Medicaid programs and Medicare. These reserves are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability which is included in accrued expenses on the consolidated balance sheets. For Medicare, we also estimate the number of patients in the prescription drug coverage gap for whom we will owe an additional liability under the Medicare Part D program. Our liability for these rebates consists of invoices received for claims from prior quarters that have not been paid or for which an invoice has not yet been received, estimates of claims for the current quarter, and estimated future claims that will be made for product that has been recognized as revenue, but which remains in the distribution channel inventories at the end of each reporting period.

**Other Incentives:** Other incentives which we offer include voluntary co-pay assistance programs, which are intended to provide financial assistance to qualified commercially-insured patients with prescription drug co-payments required by payors. The calculation of the accrual for co-pay assistance is based on an estimate of claims and the cost per claim that we expect to receive for product that has been recognized as revenue, but remains in the distribution channel inventories at the end of each reporting period. The adjustments are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability which is included as a component of accrued expenses on the consolidated balance sheets.

**Product Returns:** Consistent with industry practice, we generally offer customers a limited right of return for product that has been purchased from us. We estimate the amount of our product sales that may be returned by our customers and record this estimate as a reduction of revenue in the period the related product revenue is recognized. We estimate product return liabilities using available industry data and our own sales information, including our visibility into the inventory remaining in the distribution channel.

Our limited return policy allows for eligible returns of COPIKTRA for credit under the following circumstances:

- Receipt of damaged product;
- Shipment errors that were a result of an error by us;
- Expired product that is returned during the period beginning three months prior to the product's expiration and ending six months after the expiration date;
- Product subject to a recall; and
- Product that we, at our sole discretion, have specified can be returned for credit.

We have not received any returns to date and believe that returns of our product will be minimal.

If taxes should be collected from customers relating to product sales and remitted to governmental authorities, they will be excluded from product revenue. We expense incremental costs of obtaining a contract when incurred, if the expected amortization period of the asset that we would have recognized is one year or less. However, no such costs were incurred during the year ended December 31, 2018.

## Table of Contents

Exclusive Licenses of Intellectual Property - We may enter into collaboration and licensing arrangements for research and development, manufacturing, and commercialization activities with collaboration partners for the development and commercialization of our product candidates, which have components within the scope of ASC 606. The arrangements generally contain multiple elements or deliverables, which may include (i) licenses, or options to obtain licenses, to our intellectual property, (ii) research and development activities performed for the collaboration partner, (iii) participation on joint steering committees, and (iv) the manufacturing of commercial, clinical or preclinical material. Payments pursuant to these arrangements typically include non-refundable, upfront payments, milestone payments upon the achievement of significant development events, research and development reimbursements, sales milestones, and royalties on future product sales. The amount of variable consideration is constrained until it is probable that the revenue is not at a significant risk of reversal in a future period. The contracts into which we enter generally do not include significant financing components.

In determining the appropriate amount of revenue to be recognized as we fulfill our obligations under each of our collaboration and license agreements, we perform the following steps: (i) identification of the promised goods or services in the contract within the scope of ASC 606; (ii) determination of whether the promised goods or services are performance obligations including whether they are distinct in the context of the contract; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue when (or as) we satisfy each performance obligation. As part of the accounting for these arrangements, we must use significant judgment to determine: a) the number of performance obligations based on the determination under step (ii) above; b) the transaction price under step (iii) above; c) the stand-alone selling price for each performance obligation identified in the contract for the allocation of transaction price in step (iv) above; and d) the measure of progress in step (v) above. We use judgment to determine whether milestones or other variable consideration, except for royalties, should be included in the transaction price as described further below.

If a license to our intellectual property is determined to be distinct from the other promises or performance obligations identified in the arrangement, we recognize revenue from non-refundable, upfront fees allocated to the license when the license is transferred to the customer and the customer is able to use and benefit from the license. In assessing whether a promise or performance obligation is distinct from the other elements, we consider factors such as the research, development, manufacturing and commercialization capabilities of the collaboration partner and the availability of its associated expertise in the general marketplace. In addition, we consider whether the collaboration partner can benefit from a promise for its intended purpose without the receipt of the remaining elements, whether the value of the promise is dependent on the unsatisfied promise, whether there are other vendors that could provide the remaining promise, and whether it is separately identifiable from the remaining promise. For licenses that are combined with other promises, we utilize judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue. We evaluate the measure of progress as of each reporting period and, if necessary, adjust the measure of performance and related revenue recognition. The measure of progress, and thereby periods over which revenue should be recognized, is subject to estimates by management and may change over the course of the arrangement. Such a change could have a material impact on the amount of revenue we record in future periods.

**Customer Options:** If an arrangement is determined to contain customer options that allow the customer to acquire additional goods or services such as research and development services or manufacturing services, the goods and services underlying the customer options are not considered to be performance obligations at the inception of the arrangement; rather, such goods and services are contingent on exercise of the option, and the associated option fees are not included in the transaction price. We evaluate customer options for material rights or options to acquire additional goods or services for free or at a discount. If a customer option is determined to represent a material right, the material right is recognized as a separate performance obligation at the outset of the arrangement. We allocate the transaction price to material rights based on the relative standalone selling price, which is determined based on the identified discount and the estimated probability that the customer will exercise the option. Amounts allocated to a material right are not recognized as revenue until, at the earliest, the option is exercised.

**Milestone Payments:** At the inception of each arrangement that includes milestone payments, we evaluate whether the milestones are considered probable of being achieved and estimate the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not

## Table of Contents

occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the control of us or the licensee, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. We evaluate factors such as the scientific, clinical, regulatory, commercial, and other risks that must be overcome to achieve the respective milestone in making this assessment. There is considerable judgment involved in determining whether it is probable that a significant revenue reversal would not occur. At the end of each subsequent reporting period, we reevaluate the probability of achievement of all milestones subject to constraint and, if necessary, adjust our estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect revenues and earnings in the period of adjustment.

**Royalties:** For arrangements that include sales-based royalties, including milestone payments based on a level of sales, and the license is deemed to be the predominant item to which the royalties relate, we recognize revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, we have not recognized any royalty revenue resulting from any of our licensing arrangements.

**Collaborative Arrangements:** Contracts are considered to be collaborative arrangements when they satisfy the following criteria defined in ASC 808, Collaborative Arrangements: (i) the parties to the contract must actively participate in the joint operating activity and (ii) the joint operating activity must expose the parties to the possibility of significant risks and rewards, based on whether or not the activity is successful. Payments received from or made to a partner that are the result of a collaborative relationship with a partner, instead of a customer relationship, such as co-development activities, are recorded as a reduction or increase to research and development expense, respectively.

### 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.



## Table of Contents

### 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 prior to December 31, 2017. Therefore, for annual periods ending on or before December 31, 2017, we used the historical volatility of a representative group of public biotechnology and life sciences companies with similar characteristics to us. The computation of expected volatility for these annual periods 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. As of the first quarter of 2018, there was sufficient company-specific historical and implied volatility information. As such, for the annual period ending December 31, 2018, the computation of expected volatility is based only on the historical volatility of our common stock. 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, 2018, there was approximately \$17.5 million of unrecognized stock based compensation related to stock options, which are expected to be recognized over a weighted average period of 3.7 years. As of December 31, 2018, there was approximately \$1.2 million of unrecognized stock-based compensation related to RSUs, which are expected to be recognized over a weighted-average period of 1.87 years. See Notes 2 and

## Table of Contents

10 to our consolidated financial statements located in this Annual Report on Form 10-K for further discussion of share based compensation.

### Accounts Receivable, Net

Accounts receivable, net relates to amounts due from customers, net of applicable revenue reserves. Accounts receivable are typically due within 31 days. We analyze accounts that are past due for collectability and provide an allowance for receivables when collection becomes doubtful. Given the nature and limited history of collectability of our accounts receivable, an allowance for doubtful accounts is not deemed necessary at December 31, 2018.

### Inventory

We capitalize inventories manufactured in preparation for initiating sales of a product candidate when the related product candidate is considered to have a high likelihood of regulatory approval and the related costs are expected to be recoverable through sales of the inventories. In determining whether or not to capitalize such inventories, we evaluate, among other factors, information regarding the product candidate's safety and efficacy, the status of regulatory submissions and communications with regulatory authorities and the outlook for commercial sales, including the existence of current or anticipated competitive drugs and the availability of reimbursement. In addition, we evaluate risks associated with manufacturing the product candidate, including the ability of our third-party suppliers to complete the validation batches, and the remaining shelf life of the inventories. Costs associated with manufacturing product candidates prior to satisfying the inventory capitalization criteria are charged to research and development expense as incurred.

We value our inventories at the lower of cost or estimated net realizable value. We determine the cost of our inventories, which includes amounts related to materials and manufacturing overhead, on a first-in, first-out basis. We perform an assessment of the recoverability of capitalized inventory during each reporting period, and we write down any excess and obsolete inventories to their estimated realizable value in the period in which the impairment is first identified. Such impairment charges, should they occur, are recorded within costs of revenues. The determination of whether inventory costs will be realizable requires estimates by management. If actual market conditions are less favorable than projected by management, additional write-downs of inventory may be required which would be recorded as a cost of revenues in the consolidated statements of operations and comprehensive loss.

Shipping and handling costs for product shipments are recorded as incurred in costs of revenues along with costs associated with manufacturing the product, and any inventory write-downs.

### Intangible Assets

We record finite-lived intangible assets related to certain capitalized milestone payments at their fair value. These assets are amortized on a straight-line basis over their remaining useful lives, which are estimated based on the shorter

of the remaining underlying patent life or the estimated useful life of the underlying product.

We assess our finite-lived intangible assets for impairment if indicators are present or changes in circumstance suggest that impairment may exist. Events that could result in an impairment include the receipt of additional clinical or nonclinical data regarding one of our drug candidates or a potentially competitive drug candidate, changes in the clinical development program for a drug candidate, or new information regarding potential sales for the drug. If impairment indicators are present or changes in circumstance suggest that impairment may exist, we perform a recoverability test by comparing the sum of the estimated undiscounted cash flows of each finite-lived intangible asset to its carrying value on the consolidated balance sheets. If the undiscounted cash flows used in the recoverability test are less than the carrying value, we would determine the fair value of the finite-lived intangible asset and recognize an impairment loss if the carrying value of the finite-lived intangible asset exceeds its fair value.

85

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Table of Contents

## 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.

	Year Ended December 31,		
	2018	2017	2016
Revenue:			
Product revenue, net	1,718	—	—
License revenue	25,000	—	—
Total revenue	26,718	—	—
Operating expenses:			
Costs of revenues, excluding amortization of acquired intangible assets	\$ 165	\$ —	\$ —
Research and development	43,648	46,423	19,779
Selling, general and administrative	77,265	21,381	17,223
Amortization of acquired intangible assets	423	—	—
Total operating expenses	121,501	67,804	37,002
Loss from operations	(94,783)	(67,804)	(37,002)
Other income	25,556	—	—
Interest income	2,603	561	562
Interest expense	(5,810)	(559)	—
Net loss	\$ (72,434)	\$ (67,802)	\$ (36,440)

## Comparison of the Year Ended December 31, 2018 to the Year Ended December 31, 2017

**Product revenue, net.** We began commercial sales of COPIKTRA within the United States in September 2018, following receipt of FDA marketing approval on September 24, 2018. For the year ended December 31, 2018 (2018 Period) we recorded approximately \$1.7 million of net product revenue. We had no product revenue during the year ended December 31, 2017 (2017 Period).

**License revenue.** Revenue for the 2018 Period was \$25.0 million and was related to upfront payments pursuant to the license and collaboration agreements with Yakult and CSPC. We had no license revenue during the 2017 Period.

**Costs of revenues, excluding amortization of acquired intangible assets.** Costs of revenues, excluding amortization of acquired intangible assets (cost of revenues) of approximately \$0.2 million for the 2018 Period, consisted of costs associated with the manufacturing of COPIKTRA, royalties owed to Infinity on such sales, and certain period costs. We expensed the manufacturing costs of COPIKTRA as research and development expenses in the periods prior to July 1, 2018. In the third quarter of 2018, we began capitalizing inventory costs for COPIKTRA manufactured in preparation for our launch in the United States based on our evaluation of, among other factors, the status of the COPIKTRA NDA in the United States and the ability of our third-party suppliers to successfully manufacture commercial quantities of COPIKTRA. Certain of the costs of COPIKTRA units recognized as revenue during the 2018 Period were expensed prior to the September 2018 FDA marketing approval and, therefore, are not included in cost of sales during this period. We had no cost of revenues during the 2017 Period.

Research and development expense. Research and development expense for the 2018 Period was \$43.6 million compared to \$46.4 million for the 2017 Period. The \$2.8 million decrease from the 2017 Period to the 2018 Period was primarily related to a decrease of \$6.0 million in license fees related to a one-time milestone payment pursuant to the Infinity license agreement that was recognized in the 2017 Period and a decrease of approximately \$3.2 million in consulting fees, partially offset by increases of \$4.0 million in personnel related costs, including non-cash stock-based compensation, and \$1.9 million in CRO expense for outsourced biology, development and clinical services, which includes our clinical trial costs, and approximately \$0.5 million of other costs.

Table of Contents

**Selling, general and administrative expense.** Selling, general and administrative expense for the 2018 Period was \$77.3 million compared to \$21.4 million for the 2017 Period. The increase of \$55.9 million from the 2017 Period to the 2018 Period primarily resulted from an increase in personnel related costs, including non-cash stock-based compensation, of \$26.9 million, primarily related to the hiring and staffing of our sales and commercial teams, an increase in consulting and professional fees of \$24.4 million, primarily related to the support of the commercial launch preparation activities, and an increase in travel and other costs of \$4.6 million.

**Amortization of acquired intangible assets.** Amortization of acquired intangible assets for the 2018 Period of approximately \$0.4 million was related to the COPIKTRA finite-lived intangible asset which we recognized and began amortizing in September 2018. There was no amortization of acquired intangible assets in the 2017 Period.

**Other income.** Other income for the 2018 Period of approximately \$25.6 million was related to the mark-to-market adjustment of the bifurcated conversion option derivative liability related to the Notes. There was no mark-to-market adjustment or any other income in the 2017 Period.

**Interest income.** Interest income for the 2018 Period was \$2.6 million compared to \$0.6 million for the 2017 Period. The increase of \$2.0 million from the 2017 Period to the 2018 period is primarily due to higher investment cost basis and higher interest rates on investments.

**Interest expense.** Interest expense for the 2018 Period was \$5.8 million compared to \$0.6 million for the 2017 Period. The increase of \$5.2 million was due to a higher principal balance and higher interest rates on our loan and security agreement with Hercules, an increase in the number of days the loan with Hercules was outstanding in the 2018 Period compared to the 2017 Period, and the issuance of the Notes in October 2018.

**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 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 \$0.2 million in stock-based compensation and other expenses.

**Selling, general and administrative expense.** Selling, 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 \$0.2 million. 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 \$0.6 million 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.

## LIQUIDITY AND CAPITAL RESOURCES

### Sources of liquidity

We have financed our operations to date primarily through public offerings of our common stock, sales of common stock under our at-the market equity offering programs, our loan and security agreement executed with Hercules in March 2017, as amended, the upfront payments under our license and collaboration agreements with

87

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Table of Contents

Yakult and CSPC and the issuance in October 2018 of \$150.0 million aggregate principal amount of 5.00% Convertible Senior Notes due 2048. With the commercial launch of COPIKTRA in the United States in September 2018, we have recently begun financing a portion of our operations through product revenue.

As of December 31, 2018, we had \$249.7 million in cash, cash equivalents and short-term investments. We primarily invest our cash, cash equivalents and investments in U.S. Government money market funds and corporate bonds and commercial paper of publicly traded companies.

COPIKTRA is our only approved product and our business currently depends heavily on its successful commercialization. Successful commercialization of an approved product is an expensive and uncertain process. Risks and uncertainties include those identified under Item 1A. Risk Factors, in this Annual Report on Form 10-K.

## 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,		
	2018	2017	2016
Net cash (used in) provided by:			
Operating activities	\$ (74,515)	\$ (57,310)	\$ (29,484)
Investing activities	(138,377)	43,953	36,927
Financing activities	261,162	63,184	(5)
Increase in cash, cash equivalents and restricted cash	\$ 48,270	\$ 49,827	\$ 7,438

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 \$17.2 million increase in cash used in operating activities for the 2018 Period compared to the 2017 Period was primarily due to an increase in selling, general, and administrative expenses related to the hiring and staffing of our sales and commercial teams as well as an increase in consulting and professional fees primarily related to the support of the commercial launch preparation activities. 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.

Investing activities. The cash used in investing activities for the 2018 Period relates to the net purchases of investments of \$115.0 million, the acquisition of the COPIKTRA finite-lived intangible asset of \$22.0 million and net purchases of property and equipment of approximately \$1.4 million. 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.

Financing activities. The cash provided by financing activities for the 2018 Period primarily represents \$145.3 million in net proceeds received from the issuance of our 5.00% Convertible Senior Notes due 2048, \$81.2 million in net proceeds from the sales of our common stock under the Underwriting Agreement and Purchase Agreement described below, \$24.3 million in net proceeds received under our at-the-market equity offering program (ATM), \$9.9 million in net proceeds received from our loan and security agreement executed with Hercules, and approximately \$0.8 million related to stock option exercises, offset by the payment of approximately \$0.3 million of issuance costs related to a sale of our common stock during December 2017. The cash provided by financing activities for the 2017 Period primarily represents \$24.7 million in net proceeds received from an underwritten offering 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 \$0.4 million received from the exercise of stock options, offset by approximately \$0.1 million of deferred financing costs.

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 ongoing research and development

88

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Table of Contents

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 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), on March 6, 2018, we entered into the Second Amendment to the Original Loan Agreement (the Second Amendment) and on October 11, 2018, we entered into the Third Amendment to the Original Loan Agreement (the Third Amendment, and the Original Loan Agreement as amended by the First Amendment, the Second Amendment and Third 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 was \$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. In June 2018, we borrowed an additional \$10.0 million as a Term E Loan Advance.

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, (i) any failure by us to make any payments of principal or interest under the Amended Loan Agreement, promissory notes or other loan documents, (ii) any breach or default in the performance of any covenant under the Amended Loan Agreement, (iii) any making of false or misleading representations or warranties in any material respect, (iv) our insolvency or bankruptcy, (v) certain attachments or judgments on the assets of Verastem, Inc., or (vi) the occurrence of any material default under certain agreements or obligations of ours involving indebtedness, or (vii) 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.

## Table of Contents

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.

On March 30, 2017, 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 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, 2018, we sold 11,518,354 shares under the 2017 ATM Program for net proceeds of approximately \$47.3 million (after deducting commissions and other offering expenses).

On May 16, 2018, we entered into an underwriting agreement with Cantor relating to the underwritten offering of 7,777,778 shares of our common stock (the Underwriting Agreement). Cantor agreed to purchase the shares of our common stock pursuant to the Underwriting Agreement at a price of \$4.31 per share. In addition, we granted Cantor an option to purchase, at the public offering price less any underwriting discounts and commissions, an additional 1,166,666 shares of our common stock, exercisable for 30 days from the date of the prospectus supplement. The option was exercised by Cantor on May 23, 2018. The aggregate proceeds from Cantor, net of underwriting discounts and offering costs, were approximately \$38.3 million.

On June 14, 2018, we entered into a purchase agreement with Consonance Capital Master Account L.P. and P Consonance Opportunities Ltd. (collectively, Consonance) relating to the registered offering of 7,166,666 shares of our common stock at a price of \$6.00 per share (the Purchase Agreement). The aggregate proceeds from Consonance, net of offering costs, were approximately \$42.9 million.

On October 17, 2018, we closed a registered direct public offering of \$150.0 million aggregate principal amount of our 5.00% Convertible Senior Notes due 2048 (the Notes), for net proceeds of \$145.3 million. The Notes are governed by the terms of a base indenture for senior debt securities (the Base Indenture), as supplemented by the first supplemental indenture thereto (the Supplemental Indenture and together with the Base Indenture, the Indenture), each dated October 17, 2018, by and between us and Wilmington Trust, National Association, as trustee. The Notes are senior unsecured obligations of us and bear interest at a rate of 5.00% per annum, payable semi-annually in arrears on May 1 and November 1 of each year, beginning on May 1, 2019. The Notes will mature on November 1, 2048, unless earlier repurchased, redeemed or converted in accordance with their terms.

The Notes are convertible into shares of our common stock, par value \$0.0001 per share, together, if applicable, with cash in lieu of any fractional share, at an initial conversion rate of 139.5771 shares of common stock per \$1,000 principal amount of the Notes, which corresponds to an initial conversion price of approximately \$7.16 per share of common stock and represents a conversion premium of approximately 15.0% above the last reported sale price of our common stock of \$6.23 per share on October 11, 2018. Upon conversion, converting noteholders will be entitled to receive accrued interest on their converted Notes. To the extent we have insufficient authorized but unissued shares to settle conversions in shares of common stock, we would be required to settle the deficiency in cash.

We will have the right, exercisable at our option, to cause all Notes then outstanding to be converted automatically if the "Daily VWAP" (as defined in the Indenture) per share of our common stock equals or exceeds 130% of the conversion price on each of at least 20 VWAP Trading Days, whether or not consecutive, during any 30 consecutive VWAP Trading Day period commencing on or after the date we first issued the Notes.

The conversion rate is subject to adjustment from time to time upon the occurrence of certain events, including, but not limited to, the issuance of stock dividends and payment of cash dividends, but will not be adjusted for any accrued and unpaid interest.

We assessed all terms and features of the Notes in order to identify any potential embedded features that would require bifurcation. As part of this analysis, we assessed the economic characteristics and risks of the Notes, including the conversion, put and call features. Per the terms of the Indenture, upon conversion of the Notes, a portion of the principal may be settled in cash until the date upon which our stockholders approve an increase in the number of authorized shares of common stock, or the Authorized Share Effective Date. In consideration of this provision, we concluded the conversion feature required bifurcation as a derivative. The fair value of the

Table of Contents

conversion feature derivative was determined based on the difference between the fair value of the Notes with the conversion option and the fair value of the Notes without the conversion option. We determined that the fair value of the derivative upon issuance of the Notes was \$51.5 million and recorded this amount as a derivative liability and the offsetting amount as a debt discount as a reduction to the carrying value of the Notes on the closing date, or October 17, 2018.

On December 18, 2018, the Authorized Share Effective Date was achieved as our stockholders approved an increase in the number of authorized shares of common stock. Following this approval, no portion of the Notes are settleable in cash upon conversion. As such, we determined that the conversion feature no longer met the definition of a derivative following the increase in the number of authorized shares of common stock. As of December 18, 2018, we determined the fair value of the conversion feature was \$25.9 million. We recorded the change in the fair value of the conversion feature for the period from October 17, 2018 to December 18, 2018 of \$25.6 million as other income on the consolidated statements of operations and comprehensive loss. As of December 18, 2018, the fair value of the conversion option was reclassified to additional paid-in capital on the consolidated balance sheets as it qualified for a scope exception from derivative accounting.

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:

- commercialize COPIKTRA;
- continue our ongoing clinical trials, including with COPIKTRA 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 commercialization efforts; and

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

We expect our existing cash, cash equivalents and short-term investments will be sufficient to fund our obligations for at least the next twelve months from the date of filing of this Annual Report on Form 10-K. We have based this estimate on assumptions that may prove to be wrong and we could use our available capital resources sooner than we currently expect. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates, and the extent to which we may enter into collaborations with third parties for development and commercialization of our product candidates, we are unable to estimate the amounts of increased capital outlays and operating expenses associated with completing the development of our current product candidates. Our future capital requirements will depend on many factors, including:

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

Table of Contents

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, 2018:

(in thousands)	Total	2019	2020 - 2021	2022 - 2023	Thereafter
Operating lease obligations	\$ 6,348	\$ 716	\$ 1,991	\$ 2,103	\$ 1,538
Amended Loan Agreement	25,000	5,930	19,070	—	—
5.00% Convertible Senior Notes	150,000	—	—	—	150,000
License agreements (1)	—	—	—	—	—

(1) As discussed in Note 15 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.

**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, 2018, we had federal and state net operating loss carryforwards of \$322.4 million and \$322.2 million, respectively, which are available to reduce future taxable income. We also had federal and state tax credits of \$16.7 million and \$2.0 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, 2018, we recorded a 100% valuation allowance against our net operating loss and tax credit carryforwards of \$98.3 million, as we believe it is more likely than not that the tax benefits will not be fully realized. In the future, if we determine that a portion or all of the tax benefits associated with our tax carryforwards will be realized, net income would increase in the period of determination.

Table of Contents

## RECENTLY ADOPTED ACCOUNTING STANDARDS

In May 2017, the Financial Accounting Standards Board (FASB) issued Accounting Standard 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 was effective for annual and interim periods beginning after December 15, 2017, with early adoption permitted. We adopted this standard prospectively effective January 1, 2018. The adoption of this ASU did not have an effect on our consolidated financial statements or related disclosures.

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 was effective for annual and interim periods beginning after December 15, 2017, with early adoption permitted. We adopted this standard effective January 1, 2018. Upon adoption of ASU 2016-18, we applied the retrospective transition method for each period presented and included approximately \$162,000 of restricted cash in the beginning-of-period and end-of-period cash, cash equivalents and restricted cash balance reflected in the consolidated statements of cash flows for the year ended December 31, 2017. A reconciliation of cash, cash equivalents and restricted cash for each period presented is provided in Note 2 to the consolidated financial statements.

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 was effective for annual and interim periods beginning after December 15, 2017, with early adoption permitted. We adopted this standard effective January 1, 2018. The adoption of this ASU did not have an effect on our consolidated financial statements or related disclosures.

In May 2014, the FASB issued ASU 2014-09, Revenue from Contracts with Customers (Topic 606) which amends the guidance for accounting for revenue from contracts with customers. This ASU supersedes the revenue recognition requirements in ASC Topic 605, Revenue Recognition. In 2015 and 2016, the FASB issued additional ASUs related to ASC 606 that delayed the effective date of the guidance and clarified various aspects of the new revenue guidance, including principal versus agent considerations, identifying performance obligations, and licensing, and they include other improvements and practical expedients. We adopted this new standard on January 1, 2018 using the full retrospective method. There was no change to our consolidated financial statements as a result of the adoption.

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 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.

## Table of Contents

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 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 short-term investments of \$249.7 million and \$86.7 million as of December 31, 2018 and 2017, respectively, consisting of cash, U.S. Government money market funds, 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, 2018, an immaterial amount of our total liabilities was denominated in currencies other than the functional currency.

As of December 31, 2018, we have borrowed \$25.0 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, 2018.

The Notes bear interest at a fixed rate and therefore have minimal exposure to changes in interest rates; however, because these interest rates are fixed, we may be paying a higher interest rate, relative to market, in the future if our credit rating improves or other circumstances change.

### 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 39 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

## Table of Contents

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.

### 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, 2018.

The effectiveness of our internal control over financial reporting as of December 31, 2018 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 during the fiscal quarter ended December 31, 2018 that has materially affected, or is reasonably likely to materially affect, the Company's internal control over financial reporting.

## Table of Contents

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, 2018, 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, 2018, 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 consolidated balance sheets of Verastem, Inc. as of December 31, 2018 and 2017, and 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, 2018, and the related notes of the Company and our report dated March 12, 2019 expressed unqualified opinion thereon.

### 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 12, 2019



Table of Contents

Item 9B. Other Information

None.

97

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Table of Contents

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS, AND CORPORATE GOVERNANCE

Information regarding our directors, including the audit committee and audit committee financial experts, and executive officers and compliance with Section 16(a) of the Exchange Act will be included in our 2019 Proxy Statement and is incorporated herein by reference.

We have adopted a Code of Business Conduct and Ethics for all of our directors, officers and employees as required by Nasdaq governance rules and as defined by applicable SEC rules. Stockholders may locate a copy of our Code of Business Conduct and Ethics on our website at [www.verastem.com](http://www.verastem.com) or request a copy without charge from:

Verastem, Inc.

Attention: Investor Relations

117 Kendrick St., Suite 500

Needham, MA 02494

We will post to our website any amendments to the Code of Business Conduct and Ethics and any waivers that are required to be disclosed by the rules of either the SEC or Nasdaq.

ITEM 11. EXECUTIVE COMPENSATION

The information required by this Item 11 of Form 10-K regarding executive compensation will be included in our 2019 Proxy Statement and is incorporated herein by reference.

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

The information required by this Item 12 of Form 10-K regarding security ownership of certain beneficial owners and management will be included in our 2019 Proxy Statement and is incorporated herein by reference.

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

The information required by this Item 13 of Form 10-K regarding certain relationships and related transactions and director independence will be included in our 2019 Proxy Statement and is incorporated herein by reference.

ITEM 14. PRINCIPAL ACCOUNTANT FEES AND SERVICES

The information required by this Item 14 of Form 10-K regarding principal accountant fees and services will be included in our 2019 Proxy Statement and is incorporated herein by reference.

Table of Contents

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.

Table of Contents

EXHIBIT INDEX

Exhibit number	Description of exhibit
3.1*	<u>Restated Certificate of Incorporation of the Registrant</u>
3.2*	<u>Certificate of Amendment to the Restated Certificate of Incorporation of the Registrant</u>
3.3	<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>
4.2	<u>Indenture, dated as of October 17, 2018, by and between the Registrant and Wilmington Trust, National Association (incorporated by reference to Exhibit 4.1 to Form 8-K filed by the Registrant on October 17, 2018)</u>
4.3	<u>First Supplemental Indenture, dated as of October 17, 2018, by and between the Registrant and Wilmington Trust, National Association (incorporated by reference to Exhibit 4.2 to Form 8-K filed by the Registrant on October 17, 2018)</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 (incorporated by reference to Exhibit 10.1 of the Registrant's Current Report on Form 8-K filed by the Registrant on December 20, 2018)</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 (incorporated by reference to Exhibit 10.4 of the Registrant's Annual Report on Form 10-K filed by the Registrant on March 13, 2018)</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 (incorporated by reference to Exhibit 10.6 of the Registrant's Annual Report on Form 10-K filed by the Registrant on March 13, 2018)</u>

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10.7# Form of Restricted Stock Unit Agreement under 2012 Incentive Plan (incorporated by reference to Exhibit 10.16 to Amendment No. 3 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on January 13, 2012)

10.8# 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)

100

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Table of Contents

- 10.9# Form of Restricted Stock Unit Agreement under Amended and Restated 2012 Incentive Plan (incorporated by reference to Exhibit 10.9 of the Registrant’s Annual Report on Form 10-K filed by the Registrant on March 13, 2018)
- 10.10# 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)
- 10.11# Form of Inducement Award Nonstatutory Stock Option Agreement (incorporated by reference to Exhibit 10.11 of the Registrant’s Annual Report on Form 10-K filed by the Registrant on March 13, 2018)
- 10.12# Form of Inducement Award Restricted Stock Unit Agreement (incorporated by reference to Exhibit 4.3 of the Registrant’s Quarterly Report on Form 10-Q for the quarter ended September 30, 2018, filed by the Registrant with the Securities and Exchange Commission on November 7, 2018)
- 10.13# 2018 Employee Stock Purchase Plan (incorporated by reference to Exhibit 10.2 of the Registrant’s Current Report on Form 8-K filed by the Registrant on December 20, 2018)
- 10.14# 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)
- 10.15# 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)
- 10.16 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)
- 10.17 First Amendment of Lease Agreement, dated February 15, 2018, between the Registrant and 117 Kendrick DE, LLC, as successor-in-interest to Intercontinental Fund III 117 Kendrick Street, LLC (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed by the Registrant on May 3, 2018)
- 10.18# 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)
- 10.19† 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)
- 10.20# 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)
- 10.21† 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)
- 10.22# 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)



Table of Contents

- 10.23# 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)
- 10.24# 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)
- 10.25# Employment Agreement between the Registrant and Robert Gagnon, effective August 28, 2018 (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed by the Registrant on August 29, 2018)
- 10.26# 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)
- 10.27† 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)
- 10.28 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)
- 10.29 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)
- 10.30 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. (incorporated by reference to Exhibit 10.28 to the Annual Report on Form 10-K filed by the Registrant on March 13, 2018)
- 10.31 Third Amendment to Loan and Security Agreement, as amended, with Hercules Capital, Inc., as administrative agent, and the Lenders from time to time party thereto (incorporated by reference to Exhibit 10.1 to Form 8-K filed by the Registrant on October 11, 2018)
- 10.32# Employment Agreement between the Registrant and Joseph Lobacki, dated January 3, 2018 (incorporated by reference to Exhibit 10.29 of the Registrant's Annual Report on Form 10-K filed by the Registrant on March 13, 2018)
- 10.33† License and Collaboration Agreement, dated September 25, 2018, between Verastem, Inc. and CSPC Pharmaceutical Group Limited (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed by the Registrant on November 7, 2018)
- 10.34† License and Collaboration Agreement, dated June 5, 2018, between Verastem, Inc. and Yakult Honsha Co., Ltd. (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed by the Registrant on August 8, 2018)



10.35 Consulting Agreement, dated October 3, 2018, between Verastem, Inc. and Louise Phanstiel. (incorporated by reference to Exhibit 10.2 to the Quarterly Report on Form 10-Q filed by the Registrant on November 7, 2018)

21.1\* Subsidiaries of the Registrant

102

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Table of Contents

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 12, 2019 (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 SEC.

‡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.



Table of Contents

## 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 12th day of March 2019.

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 12, 2019
/s/ Robert Gagnon Robert Gagnon	Chief Financial Officer (Principal financial and accounting officer)	March 12, 2019
/s/ Timothy Barberich Timothy Barberich	Director	March 12, 2019
/s/ Gina Consylman Gina Consylman	Director	March 12, 2019
/s/ Michael Kauffman, M.D., Ph.D. Michael Kauffman, M.D., Ph.D.	Director	March 12, 2019
/s/ Alison Lawton Alison Lawton	Director	March 12, 2019
/s/ Eric Rowinsky, M.D. Eric Rowinsky, M.D.	Director	March 12, 2019
/s/ Brian Stuglik, R.Ph, Brian Stuglik, R.Ph.	Director	March 12, 2019
/s/ Bruce Wendel Bruce Wendel	Director	March 12, 2019



Table of Contents

Verastem, Inc.

CONSOLIDATED FINANCIAL STATEMENTS

CONTENTS

<u>Report of Independent Registered Public Accounting Firm</u>	F 2
Consolidated Financial Statements	
<u>Consolidated Balance Sheets</u>	F 3
<u>Consolidated Statements of Operations and Comprehensive Loss</u>	F 4
<u>Consolidated Statements of Stockholders' Equity</u>	F 5
<u>Consolidated Statements of Cash Flows</u>	F 6
<u>Notes to Consolidated Financial Statements</u>	F 7

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Table of Contents

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, 2018 and 2017, and 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, 2018, 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 consolidated financial position of the Company at December 31, 2018 and 2017, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2018, 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, 2018, 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 12, 2019 expressed an unqualified opinion thereon.

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 12, 2019





Table of Contents

Verastem, Inc.

## CONSOLIDATED BALANCE SHEETS

(in thousands, except per share amounts)

	December 31,	
	2018	2017
Assets		
Current assets:		
Cash and cash equivalents	\$ 129,867	\$ 82,176
Short-term investments	119,786	4,496
Accounts receivable, net	306	—
Inventory	327	—
Prepaid expenses and other current assets	2,973	1,115
Total current assets	253,259	87,787
Property and equipment, net	1,369	861
Intangible assets, net	21,577	—
Other assets	1,031	1,143
Total assets	\$ 277,236	\$ 89,791
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	\$ 10,253	\$ 9,186
Accrued expenses	21,108	7,942
Current portion of long-term debt	5,716	—
Total current liabilities	37,077	17,128
Non-current liabilities:		
Long-term debt	19,506	14,828
Convertible senior notes	95,231	—
Other non-current liabilities	1,123	151
Total liabilities	152,937	32,107
Stockholders' equity:		
Preferred stock, \$0.0001 par value; 5,000 shares authorized, no shares issued and outstanding at December 31, 2018 and December 31, 2017, respectively	—	—
Common stock, \$0.0001 par value; 200,000 and 100,000 shares authorized, 73,806 and 50,801 shares issued and outstanding at December 31, 2018 and December 31, 2017, respectively	7	5
Additional paid-in capital	499,741	360,823
Accumulated other comprehensive income (loss)	127	(2)
Accumulated deficit	(375,576)	(303,142)
Total stockholders' equity	124,299	57,684
Total liabilities and stockholders' equity	\$ 277,236	\$ 89,791
See accompanying notes to the consolidated financial statements.		

Table of Contents

Verastem, Inc.

## CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(in thousands, except per share amounts)

	Year Ended December 31,		
	2018	2017	2016
Revenue:			
Product revenue, net	\$ 1,718	\$ —	\$ —
License revenue	25,000	—	—
Total revenue	26,718	—	—
Operating expenses:			
Costs of revenues, excluding amortization of acquired intangible assets	\$ 165	\$ —	\$ —
Research and development	43,648	46,423	19,779
Selling, general and administrative	77,265	21,381	17,223
Amortization of acquired intangible assets	423	—	—
Total operating expenses	121,501	67,804	37,002
Loss from operations	(94,783)	(67,804)	(37,002)
Other income	25,556	—	—
Interest income	2,603	561	562
Interest expense	(5,810)	(559)	—
Net loss	\$ (72,434)	\$ (67,802)	\$ (36,440)
Net loss per share—basic	\$ (1.12)	\$ (1.76)	\$ (0.99)
Net loss per share—diluted	\$ (1.37)	\$ (1.76)	\$ (0.99)
Weighted average common shares outstanding used in computing:			
Net loss per share—basic	64,962	38,422	36,988
Net loss per share—diluted	69,321	38,422	36,988
Net loss	\$ (72,434)	\$ (67,802)	\$ (36,440)
Unrealized gain (loss) on available-for-sale securities	129	(31)	(14)
Comprehensive loss	\$ (72,305)	\$ (67,833)	\$ (36,454)
See accompanying notes to the consolidated financial statements.			

F-4

Table of Contents

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, 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 units and payment of tax withholdings	49,552	—	(5)	—	—	(5)
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
	50,800,908	\$ 5	\$ 360,823	\$ (2)	\$ (303,142)	\$ 57,684

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Balance at December 31, 2017						
Net loss	—	—	—	—	(72,434)	(72,434)
Unrealized gain on available-for-sale marketable securities	—	—	—	129	—	129
Issuance of common stock resulting from follow-on offering, net of issuance costs of \$361	16,111,110	1	81,188	—	—	81,189
Issuance of common stock resulting from at-the-market transactions, net of issuance costs of \$0	6,481,475	1	24,275	—	—	24,276
Issuance of common stock resulting from exercise of stock options	412,851	—	809	—	—	809
Stock-based compensation expense	—	—	6,671	—	—	6,671
Reclassification of derivative liability to equity	—	—	25,975	—	—	25,975
Balance at December 31, 2018	73,806,344	\$ 7	\$ 499,741	\$ 127	\$ (375,576)	\$ 124,299

See accompanying notes to the consolidated financial statements.

F-5

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Table of Contents

Verastem, Inc.

## CONSOLIDATED STATEMENTS OF CASH FLOWS

(in thousands)

	Year Ended December 31,		
	2018	2017	2016
Operating activities			
Net loss	\$ (72,434)	\$ (67,802)	\$ (36,440)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation	996	556	670
Amortization of acquired intangible asset	423	—	—
Stock-based compensation expense	6,671	5,033	6,287
Amortization of deferred financing costs, debt discounts and premiums and discounts on available-for-sale marketable securities	1,814	223	(140)
Change in fair value of conversion option for convertible senior notes	(25,556)	—	—
Gain on sale of fixed assets	(79)	—	—
Changes in operating assets and liabilities:			
Accounts receivable, net	(306)	—	—
Inventory	(327)	—	—
Prepaid expenses, other current assets and other assets	(1,167)	(943)	(568)
Accounts payable	1,048	5,046	153
Accrued expenses and other liabilities	13,902	577	554
Other long-term liabilities	500	—	—
Net cash used in operating activities	(74,515)	(57,310)	(29,484)
Investing activities			
Purchases of property and equipment	(1,507)	—	(39)
Sales of property and equipment	82	—	—
Acquisition of intangible asset	(22,000)	—	—
Purchases of investments	(125,452)	(7,957)	(82,101)
Maturities of investments	10,500	51,910	119,067
Net cash (used in) provided by investing activities	(138,377)	43,953	36,927
Financing activities			
Proceeds from long-term debt, net of issuance costs	9,900	14,811	—
Deferred debt financing costs	—	(138)	—
Proceeds from issuance of convertible senior notes, net of issuance costs	145,297	—	—
Proceeds from the exercise of stock options	809	442	—
Cash used to settle restricted stock liability	—	—	(5)
Proceeds from the issuance of common stock, net	105,156	48,069	—
Net cash provided by (used in) financing activities	261,162	63,184	(5)
Increase in cash, cash equivalents and restricted cash	48,270	49,827	7,438
Cash, cash equivalents and restricted cash at beginning of period	82,338	32,511	25,073
Cash, cash equivalents and restricted cash at end of period	\$ 130,608	\$ 82,338	\$ 32,511
Supplemental disclosure			
Cash paid for interest	\$ 2,107	\$ 295	\$ —

Supplemental disclosure of non-cash financing activities

Common stock issuance costs included in accounts payable and accrued expenses

\$ 15

\$ 324

\$ —

See accompanying notes to the consolidated financial statements.

## Table of Contents

### 1. Nature of business

Verastem, Inc. (the Company) is a biopharmaceutical company focused on developing and commercializing medicines to improve the survival and quality of life of cancer patients. On September 24, 2018, the Company's first commercial product, COPIKTRA™ (duvelisib), was approved by the U.S. Food and Drug Administration (the FDA) for the treatment of patients with hematologic cancers including chronic lymphocytic leukemia (CLL) and small lymphocytic lymphoma (SLL) and follicular lymphoma (FL). Both its marketed product, COPIKTRA, and most advanced product candidate, defactinib, utilize a multi-faceted approach designed to treat cancers originating either in the blood or major organ systems. The Company is currently developing its product candidates in both preclinical and clinical studies as potential therapies for certain cancers, including leukemia, lymphoma, lung cancer, ovarian cancer, mesothelioma, and pancreatic cancer. The Company believes 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.

The Company is subject to the risks associated with other life science companies, including, but not limited to, possible failure of preclinical testing or clinical trials, competitors developing new technological innovations, market acceptance and the successful commercialization of COPIKTRA, or any of the Company's investigational product candidates following receipt of regulatory approval and protection of proprietary technology. If the Company does not successfully commercialize COPIKTRA or any of its other product candidates, it will be unable to generate product revenue or achieve profitability and may need to raise additional capital.

The Company has historical losses from operations and anticipates that it will continue to incur losses for the foreseeable future as it continues the commercialization of COPIKTRA and the research and development of its product candidates. As of December 31, 2018, the Company had cash, cash equivalents and short-term investments of \$249.7 million. The Company expects, prior to the consideration of any revenue from future potential sales of COPIKTRA, that its cash, cash equivalents and short-term investments will be sufficient to fund its development plans, U.S. commercial scale-up and other operational activities for at least twelve months from the date of issuance of these consolidated financial statements.

The Company expects to finance the future development costs of its clinical product portfolio with its existing cash, cash equivalents and short-term investments, or through strategic financing opportunities that could include, but are not limited to collaboration agreements, future offerings of its equity, or the incurrence of debt. However, there is no guarantee that any of these strategic or financing opportunities will be executed or executed on favorable terms, and some could be dilutive to existing stockholders. If the Company fails to obtain additional future capital, it may be unable to complete its planned preclinical studies and clinical trials and obtain approval of certain investigational product candidates from the FDA or foreign regulatory authorities.

### 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.

F-7

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Table of Contents

## 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 revenue recognition, including returns, rebates, and other pricing adjustments, 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.

## 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 and commercializing drugs for the treatment of cancer. All material long-lived assets of the Company reside in the United States.

## Cash, cash equivalents and restricted cash

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 funds and corporate bonds and commercial paper of publicly traded companies. Cash equivalents are reported at fair value.

The following table provides a reconciliation of cash, cash equivalents and restricted cash reported within the consolidated balance sheets that sum to the total of the same such amounts shown in the consolidated statements of cash flows (in thousands):

	December 31, 2018	December 31, 2017
Cash and cash equivalents	\$ 129,867	\$ 82,176
Restricted cash	741	162
Total cash, cash equivalents and restricted cash	\$ 130,608	\$ 82,338

Amounts included in restricted cash as of December 31, 2018 represent cash received pursuant to a funded research and development agreement with the Leukemia and Lymphoma Society (LLS) restricted for future expenditures for specific R&D studies and cash held to collateralize outstanding letters of credit provided as a security deposit for the Company's office space located in Needham, Massachusetts in the amount of approximately \$500,000 and \$241,000, respectively. Restricted cash related to LLS funded research and development agreement is included in prepaid and other current assets, while restricted cash for letters of credit are included in other assets on the consolidated balance sheets. Amounts included in restricted cash as of December 31, 2017 represent cash held to collateralize outstanding letters of credit in the amount of \$162,000 provided as a security deposit for the Company's office space located in Needham, Massachusetts and included in other assets on the consolidated balance sheets.

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

F-8

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Table of Contents

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, 2018			
	Total	Level 1	Level 2	Level 3
Financial assets				
Cash equivalents	\$ 127,689	\$ 60,092	\$ 67,597	\$ —
Short-term investments	119,786	—	119,786	—
Total financial assets	\$ 247,475	\$ 60,092	\$ 187,383	\$ —

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	\$ —

The 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 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, 2018 and 2017.

During 2018, a derivative liability was initially recorded as a result of the issuance of the 5.00% Convertible Senior Notes due 2048 (the Notes) (see note 11). The Company initially determined fair value of the liability upon issuance, and then again upon the determination that the derivative instrument met the criteria to be reclassified into equity.

The fair value measurement of the derivative liability is classified as Level 3 under the fair value hierarchy as it has been valued using unobservable inputs. These inputs include: (1) a simulated share price at the time of conversion of the Notes, (2) assumed timing of conversion of the Notes, and (3) the risk-adjusted discount rate used to present value the probability-weighted cash flows. Significant increases or decreases in any of those inputs in isolation could result in a significantly lower or higher fair value measurement.

The fair value of the derivative liability was determined using a binomial lattice model by calculating the fair value of the Notes with the conversion feature as compared to the fair value of the Notes without the conversion feature, with the difference representing the value of the conversion feature, or the derivative liability. The fair

F-9

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Table of Contents

value of the Notes with the conversion feature at issuance was assumed to equal the issuance par value of \$150.0 million with an implied discount rate of 12.1% which was determined by discounting the cash flows generated by the binomial lattice model back to the issuance par value. The fair value of the Notes without the conversion feature was calculated based on cash payment for the full par value of the Notes and was discounted by the implied discount rate of 12.1%. The fair value of the Notes with and without the conversion feature upon the Company's shareholders increasing the number of authorized shares of common stock was determined using a similar approach with an implied discount rate of 16.2%, which was determined by evaluating the increase in credit spreads of publicly traded debt over a similar time period.

The following table represents a reconciliation of the derivative liability recorded in connection with the issuance of the Notes:

January 1, 2018	\$ —
Fair value recognized upon issuance of Convertible Senior Notes	51,531
Fair value adjustment	(25,556)
Reclassification to equity	(25,975)
December 31, 2018	\$ —

## Fair Value of Financial Instruments

The fair value of the Company's long-term debt is determined using a discounted cash flow analysis with current applicable rates for similar instruments as of the consolidated balance sheet dates. The carrying value of the Company's long-term debt, including the current portion, at December 31, 2018 and 2017, was approximately \$25.2 million and \$14.8 million, respectively. At December 31, 2018 and 2017, the Company estimates that the fair value of its long-term debt, including the current portion, was approximately \$26.9 million and \$14.8 million, respectively. The fair value of the Company's long-term debt was determined using Level 3 inputs.

The fair value of the Notes was approximately \$108.1 million as of December 18, 2018, which differs from the carrying value of the Notes. The fair value of the Notes is influenced by our stock price and stock price volatility. The fair value of the Notes was determined using Level 3 inputs.

## Investments

Investments and cash equivalents consist of investments in a 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 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 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, 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.

F-10

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Table of Contents

There were no realized gains or losses on investments for the years ended December 31, 2018, 2017 or 2016. There were fourteen debt securities and five debt securities in an unrealized loss position as of December 31, 2018 and December 31, 2017, respectively. None of these investments had been in an unrealized loss position for more than 12 months as of December 31, 2018 or December 31, 2017, respectively. The fair value of these securities as of December 31, 2018 and December 31, 2017 was \$46.9 million and \$9.9 million, respectively, and the aggregate unrealized loss was immaterial. 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, 2018 and December 31, 2017, respectively.

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

	December 31, 2018			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Cash and cash equivalents:				
Cash and money market accounts	\$ 62,270	\$ —	\$ —	\$ 62,270
Corporate bonds and commercial paper (due within 90 days)	67,590	8	(1)	67,597
Total cash and cash equivalents	\$ 129,860	\$ 8	\$ (1)	\$ 129,867
Investments:				
Corporate bonds and commercial paper (due within 1 year)	\$ 119,666	\$ 132	\$ (12)	\$ 119,786
Total investments	\$ 119,666	\$ 132	\$ (12)	\$ 119,786
Total cash, cash equivalents and investments	\$ 249,526	\$ 140	\$ (13)	\$ 249,653

Table of Contents

	December 31, 2017			
	Amortized	Gross	Gross	Fair
	Cost	Unrealized	Unrealized	Value
		Gains	Losses	
Cash and cash equivalents:				
Cash and money market accounts	\$ 76,760	\$ —	\$ —	\$ 76,760
Corporate bonds and commercial paper (due within 90 days)	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

## Concentrations of credit risk and off balance sheet risk

Cash and cash equivalents, investments, and trade accounts receivable 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, 2018, 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.

As of December 31, 2018, there were two customers that cumulatively made up more than 50% of the Company's trade accounts receivable balance. The Company assesses the creditworthiness of all its customers and sets and reassesses customer credit limits to ensure collectability of any trade accounts receivable balances are assured.

For the year ended December 31, 2018, two customers, Yakult and CSPC, individually accounted for greater than 10% of the Company's total revenues.

## Property and equipment

Property and equipment consist 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 are calculated using the straight line method over the following estimated useful lives of the assets:



Laboratory equipment	5 years
Furniture	5 years
Computer equipment	3 years
Leasehold improvements	Lesser of useful life or life of lease

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

The Company reviews its long lived assets for impairment whenever events or changes in business circumstances indicate that the carrying value of assets may not be recoverable. Recoverability is measured by comparison of the asset's book value to future net undiscounted cash flows that the assets are expected to generate. If such assets are considered to be impaired, the impairment to be recognized is measured by the amount by which the book value of the assets exceed their fair value, which is measured based on the projected discounted future net cash flows arising from the assets. No material impairment losses have been recorded through December 31, 2018.

## Table of Contents

### Other assets

Other assets primarily consist of prepayments made to contract research organizations (CROs). As of December 31, 2018 and 2017, other assets were 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;
- facilities, depreciation and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, depreciation of equipment, and laboratory supplies; and
- costs associated with COPIKTRA prior to the Company concluding that regulatory approval is probable and that its net realizable value is recoverable.

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 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 applies the simplified method described in the Securities and Exchange Commission (SEC) Staff Accounting Bulletin (SAB) 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.

For annual periods ending on or before December 31, 2017, 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. As of the first quarter of 2018, the Company had sufficient company-specific historical and implied volatility information. As such, for the annual period ending December 31, 2018, the computation of expected volatility is based only on the historical volatility of the Company's common stock. The risk free interest rate is based on a treasury instrument whose term is consistent with the expected term of the stock options.

F-13

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## Table of Contents

The Company accounts for forfeitures as they occur. Stock based awards issued to non-employees, 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.

## Revenue Recognition

Effective January 1, 2018, the Company adopted Accounting Standards Codification (ASC) 606 Revenue from Contracts with Customers. This standard applies to all contracts with customers, except for contracts that are within the scope of other standards, such as leases, insurance, collaboration arrangements and financial instruments. Under ASC 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements that an entity determines are within the scope of ASC 606, the entity performs the following five step assessment: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation. The Company only applies the five-step model to contracts when it is probable that the entity will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception and once the contract is determined to be within the scope of ASC 606, the Company assesses the goods or services promised within each contract, determines which goods and services are performance obligations, and assesses whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

Product Revenue, Net – The Company sells COPIKTRA to a limited number of specialty pharmacies and specialty distributors in the United States. These customers subsequently resell COPIKTRA either directly to patients, or to community hospitals or oncology clinics with in-office dispensaries who in turn distribute COPIKTRA to patients. In addition to distribution agreements with customers, the Company also enters into arrangements with (1) certain government agencies and various private organizations (Third-Party Payers), which may provide for chargebacks or discounts with respect to the purchase of COPIKTRA, and (2) Medicare and Medicaid, which may provide for certain rebates with respect to the purchase of COPIKTRA.

The Company recognizes revenue on sales of COPIKTRA when a customer obtains control of the product, which occurs at a point in time (typically upon delivery). Product revenues are recorded at the wholesale acquisition costs, net of applicable reserves for variable consideration. Components of variable consideration include trade discounts and allowances, Third-Party Payer chargebacks and discounts, government rebates, other incentives, such as voluntary co-pay assistance, product returns, and other allowances that are offered within contracts between the Company and customers, payors, and other indirect customers relating to the Company's sale of COPIKTRA. These reserves, as detailed below, are based on the amounts earned, or to be claimed on the related sales, and are classified as reductions of accounts receivable or a current liability. These estimates take into consideration a range of possible outcomes based upon relevant factors such as, customer contract terms, information received from third parties regarding the anticipated payor mix for COPIKTRA, known market events and trends, industry data, and forecasted customer buying and payment patterns. Overall, these reserves reflect the Company's best estimates of the amount of consideration to which it is entitled with respect to sales made.

The amount of variable consideration which is included in the transaction price may be constrained and is included in the net sales price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized under contracts will not occur in a future period. The Company's analyses contemplate the application of the constraint in accordance with ASC 606. For the year ended December 31, 2018, the Company

determined a material reversal of revenue would not occur in a future period for the estimates detailed below and, therefore, the transaction price was not reduced further. Actual amounts of consideration ultimately received may differ from the Company's estimates. If actual results in the future vary from the Company's estimates, the Company will adjust these estimates, which would affect net product revenue and earnings in the period such variances become known.

F-14

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## Table of Contents

**Trade Discounts and Allowances:** The Company generally provides customers with invoice discounts on sales of COPIKTRA for prompt payment, which are explicitly stated in the Company's contracts and are recorded as a reduction of revenue in the period the related product revenue is recognized. In addition, the Company compensates its specialty distributor customers for sales order management, data, and distribution services. The Company has determined such services are not distinct from the Company's sale of COPIKTRA to the specialty distributor customers and, therefore, these payments have also been recorded as a reduction of revenue within the consolidated statements of operations and comprehensive loss through December 31, 2018.

**Third-Party Payer Chargebacks, Discounts and Fees:** The Company executes contracts with Third-Party Payers which allow for eligible purchases of COPIKTRA at prices lower than the wholesale acquisition cost charged to customers who directly purchase the product from the Company. In some cases, customers charge the Company for the difference between what they pay for COPIKTRA and the ultimate selling price to the Third-Party Payers. These reserves are established in the same period that the related revenue is recognized, resulting in a reduction of product revenue and accounts receivable, net. Chargeback amounts are generally determined at the time of resale to the qualified Third-Party Payer by customers, and the Company generally issues credits for such amounts within a few weeks of the customer's notification to the Company of the resale. Reserves for chargebacks consist of credits that the Company expects to issue for units that remain in the distribution channel inventories at the end of each reporting period that the Company expects will be sold to Third-Party Payers, and chargebacks that customers have claimed, but for which the Company has not yet issued a credit. In addition, the Company compensates certain Third-Party Payers for administrative services, such as account management and data reporting. These administrative service fees have also been recorded as a reduction of product revenue within the consolidated statements of operations and comprehensive loss through December 31, 2018.

**Government Rebates:** The Company is subject to discount obligations under state Medicaid programs and Medicare. These reserves are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability which is included in accrued expenses on the consolidated balance sheets. For Medicare, the Company also estimates the number of patients in the prescription drug coverage gap for whom the Company will owe an additional liability under the Medicare Part D program. The Company's liability for these rebates consists of invoices received for claims from prior quarters that have not been paid or for which an invoice has not yet been received, estimates of claims for the current quarter, and estimated future claims that will be made for product that has been recognized as revenue, but which remains in the distribution channel inventories at the end of each reporting period.

**Other Incentives:** Other incentives which the Company offers include voluntary co-pay assistance programs, which are intended to provide financial assistance to qualified commercially-insured patients with prescription drug co-payments required by payors. The calculation of the accrual for co-pay assistance is based on an estimate of claims and the cost per claim that the Company expects to receive for product that has been recognized as revenue, but remains in the distribution channel inventories at the end of each reporting period. The adjustments are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability which is included as a component of accrued expenses on the consolidated balance sheets.

**Product Returns:** Consistent with industry practice, the Company generally offers customers a limited right of return for product that has been purchased from the Company. The Company estimates the amount of its product sales that may be returned by its customers and records this estimate as a reduction of revenue in the period the related product revenue is recognized. The Company estimates product return liabilities using available industry data and its own sales information, including its visibility into the inventory remaining in the distribution channel.

The Company's limited return policy allows for eligible returns of COPIKTRA for credit under the following circumstances:

- Receipt of damaged product;
- Shipment errors that were a result of an error by the Company;
- Expired product that is returned during the period beginning three months prior to the product's expiration and ending six months after the expiration date;

F-15

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Table of Contents

- Product subject to a recall; and
- Product that the Company, at its sole discretion, has specified can be returned for credit.

The Company has not received any returns to date and believes that returns of its products will be minimal.

If taxes should be collected from customers relating to product sales and remitted to governmental authorities, they will be excluded from product revenue. The Company expenses incremental costs of obtaining a contract when incurred, if the expected amortization period of the asset that the Company would have recognized is one year or less. However, no such costs were incurred during the year ended December 31, 2018.

**Exclusive Licenses of Intellectual Property** - The Company may enter into collaboration and licensing arrangements for research and development, manufacturing, and commercialization activities with collaboration partners for the development and commercialization of its product candidates, which have components within the scope of ASC 606. The arrangements generally contain multiple elements or deliverables, which may include (1) licenses, or options to obtain licenses, to the Company's intellectual property, (2) research and development activities performed for the collaboration partner, (3) participation on joint steering committees, and (4) the manufacturing of commercial, clinical or preclinical material. Payments pursuant to these arrangements typically include non-refundable, upfront payments, milestone payments upon the achievement of significant development events, research and development reimbursements, sales milestones, and royalties on future product sales. The amount of variable consideration is constrained until it is probable that the revenue is not at a significant risk of reversal in a future period. The contracts into which the Company enters generally do not include significant financing components.

In determining the appropriate amount of revenue to be recognized as it fulfills its obligations under each of its collaboration and license agreements, the Company performs the following steps: (i) identification of the promised goods or services in the contract within the scope of ASC 606; (ii) determination of whether the promised goods or services are performance obligations including whether they are distinct in the context of the contract; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue when (or as) the Company satisfies each performance obligation. As part of the accounting for these arrangements, the Company must use significant judgment to determine: a) the number of performance obligations based on the determination under step (ii) above; b) the transaction price under step (iii) above; c) the stand-alone selling price for each performance obligation identified in the contract for the allocation of transaction price in step (iv) above; and d) the measure of progress in step (v) above. The Company uses judgment to determine whether milestones or other variable consideration, except for royalties, should be included in the transaction price as described further below.

If a license to the Company's intellectual property is determined to be distinct from the other promises or performance obligations identified in the arrangement, the Company recognizes revenue from non-refundable, upfront fees allocated to the license when the license is transferred to the customer and the customer is able to use and benefit from the license. In assessing whether a promise or performance obligation is distinct from the other elements, the Company considers factors such as the research, development, manufacturing and commercialization capabilities of the collaboration partner and the availability of its associated expertise in the general marketplace. In addition, the Company considers whether the collaboration partner can benefit from a promise for its intended purpose without the receipt of the remaining elements, whether the value of the promise is dependent on the unsatisfied promise, whether there are other vendors that could provide the remaining promise, and whether it is separately identifiable from the



remaining promise. For licenses that are combined with other promises, the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue. The Company evaluates the measure of progress of each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition. The measure of progress, and thereby periods over which revenue should be recognized, is subject to estimates by management and may change over the course of the arrangement. Such a change could have a material impact on the amount of revenue the Company records in future periods.

**Customer Options:** If an arrangement is determined to contain customer options that allow the customer to acquire additional goods or services such as research and development services or manufacturing services, the goods

F-16

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Table of Contents

and services underlying the customer options are not considered to be performance obligations at the inception of the arrangement; rather, such goods and services are contingent on exercise of the option, and the associated option fees are not included in the transaction price. The Company evaluates customer options for material rights or options to acquire additional goods or services for free or at a discount. If a customer option is determined to represent a material right, the material right is recognized as a separate performance obligation at the outset of the arrangement. The Company allocates the transaction price to material rights based on the relative standalone selling price, which is determined based on the identified discount and the estimated probability that the customer will exercise the option. Amounts allocated to a material right are not recognized as revenue until, at the earliest, the option is exercised.

**Milestone Payments:** At the inception of each arrangement that includes milestone payments, the Company evaluates whether the milestones are considered probable of being achieved and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the control of the Company or the licensee, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. The Company evaluates factors such as the scientific, clinical, regulatory, commercial, and other risks that must be overcome to achieve the respective milestone in making this assessment. There is considerable judgment involved in determining whether it is probable that a significant revenue reversal would not occur. At the end of each subsequent reporting period, the Company reevaluates the probability of achievement of all milestones subject to constraint and, if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect revenues and earnings in the period of adjustment.

**Royalties:** For arrangements that include sales-based royalties, including milestone payments based on a level of sales, and the license is deemed to be the predominant item to which the royalties relate, the Company recognizes revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, the Company has not recognized any royalty revenue resulting from any of its licensing arrangements.

**Collaborative Arrangements:** Contracts are considered to be collaborative arrangements when they satisfy the following criteria defined in ASC 808, Collaborative Arrangements: (i) the parties to the contract must actively participate in the joint operating activity and (ii) the joint operating activity must expose the parties to the possibility of significant risk and rewards, based on whether or not the activity is successful. Payments received from or made to a partner that are the result of a collaborative relationship with a partner, instead of a customer relationship, such as co-development activities, are recorded as a reduction or increase to research and development expense, respectively.

For a complete discussion of the Company's accounting for its license and collaboration agreements, see Note 15, License and collaboration agreements.

Accounts Receivable, Net

Accounts receivable, net consists of amounts due from customers, net of applicable revenue reserves. Accounts receivable are typically due within 31 days. The Company analyzes accounts that are past due for collectability and provides an allowance for receivables when collection becomes doubtful. Given the nature and limited history of collectability of the Company's accounts receivable, an allowance for doubtful accounts is not deemed necessary at December 31, 2018.

#### Inventory

The Company capitalizes inventories manufactured in preparation for initiating sales of a product candidate when the related product candidate is considered to have a high likelihood of regulatory approval and the related costs are expected to be recoverable through sales of the inventories. In determining whether or not to capitalize such inventories, the Company evaluates, among other factors, information regarding the product candidate's safety and efficacy, the status of regulatory submissions and communications with regulatory authorities and the outlook for commercial sales, including the existence of current or anticipated competitive drugs and the availability of

F-17

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## Table of Contents

reimbursement. In addition, the Company evaluates risks associated with manufacturing the product candidate, including the ability of the Company's third-party suppliers to complete the validation batches and the remaining shelf life of the inventories. Costs associated with manufacturing product candidates prior to satisfying the inventory capitalization criteria are charged to research and development expense as incurred.

The Company values its inventories at the lower of cost or estimated net realizable value. The Company determines the cost of its inventories, which includes amounts related to materials and manufacturing overhead, on a first-in, first-out basis. The Company performs an assessment of the recoverability of capitalized inventory during each reporting period, and it writes down any excess and obsolete inventories to their estimated realizable value in the period in which the impairment is first identified. Such impairment charges, should they occur, are recorded within cost of product revenues. The determination of whether inventory costs will be realizable requires estimates by management. If actual market conditions are less favorable than projected by management, additional write-downs of inventory may be required which would be recorded as a cost of product revenues in the consolidated statements of operations and comprehensive loss.

Shipping and handling costs for product shipments are recorded as incurred in cost of product revenues along with costs associated with manufacturing the product, and any inventory write-downs.

## Intangible Assets

The Company records finite-lived intangible assets related to certain capitalized milestone payments related to commercial products at their fair value. These assets are amortized on a straight-line basis over their remaining useful lives, which are estimated based on the shorter of the remaining underlying patent life or the estimated useful life of the underlying product.

The Company assesses its finite-lived intangible assets for impairment if indicators are present or changes in circumstance suggest that impairment may exist. Events that could result in an impairment include the receipt of additional clinical or nonclinical data regarding one of the Company's drug candidates or a potentially competitive drug candidate, changes in the clinical development program for a drug candidate, or new information regarding potential sales for the drug. If impairment indicators are present or changes in circumstance suggest that impairment may exist, the Company performs a recoverability test by comparing the sum of the estimated undiscounted cash flows of each finite-lived intangible asset to its carrying value on the consolidated balance sheets. If the undiscounted cash flows used in the recoverability test are less than the carrying value, the Company would determine the fair value of the finite-lived intangible asset and recognize an impairment loss if the carrying value of the finite-lived intangible asset exceeds its fair value.

## 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 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. Diluted net loss per common share is calculated by increasing the denominator by the weighted-average number of additional shares that could have been outstanding from securities convertible into common stock, such as stock options, restricted stock units and warrants (using the “treasury stock” method) and Notes (using the “if-converted” method), unless their effect on net loss per share is antidilutive. The effect of computing diluted net loss per common share was antidilutive for any potentially issuable shares of common stock from the conversion of stock options, restricted stock units and warrants and, as such, have been excluded from the calculation. However, under the “if-converted” method, convertible

## Table of Contents

instruments that are-in-the-money, are assumed to have been converted as of the beginning of the period or when issued, if later. Additionally, the effects of any interest expense and changes in fair value of bifurcated derivatives shall be added back to the numerator of the diluted net loss per share calculation. Refer to Note 12 for further details related to the calculation of net loss per share.

### Recently Issued Accounting Standards Updates

In November 2018, the Financial Accounting Standards Board (FASB) issued Accounting Standard Update (ASU) 2018-18, Collaborative Arrangements (Topic 808): Clarifying the Interaction between Topic 808 and Topic 606, which makes targeted improvements for collaborative arrangements to clarify that certain transactions between collaborative arrangement participants should be accounted for as revenue under Topic 606 when the collaborative arrangement participant is a customer in the context of a unit of account, adds unit of account guidance in Topic 808 to align with guidance in Topic 606, and clarifies presentation of certain revenues with a collaborative arrangement participant which are not directly related to a third party. ASU 2018-18 is effective for annual and interim periods beginning after December 15, 2019, 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.

In August 2018, the Financial Accounting Standards Board (FASB) issued Accounting Standard Update (ASU) 2018-15, Intangibles-Goodwill and Other-Internal Use Software: Customer's Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement that is a Service Contract, which aligns the requirements for capitalizing implementation costs incurred in a hosting arrangement that is a service contract with the requirements for capitalizing implementation costs incurred to develop or obtain internal-use software. ASU 2018-15 is effective for annual and interim periods beginning after December 15, 2019, 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.

In August 2018, the FASB issued ASU 2018-13, Fair Value Measurement (Topic 820): Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement, which eliminates certain disclosure requirements for fair value measurements for all entities, requires public entities to disclose certain new information and modifies some disclosure requirements. ASU 2018-13 is effective for all entities for annual and interim periods beginning after December 15, 2019. An entity is permitted to early adopt either the entire standard or only the provisions that eliminate or modify requirements. 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.

In June 2018, the FASB issued ASU 2018-07, Compensation – Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting, which expands the scope of Topic 718 to include all share-based payment transactions for acquiring goods and services to be used or consumed in its own operations by issuing share-based payment awards. ASU 2018-07 also clarifies that Topic 718 does not apply to share-based payments used to effectively provide (1) financing to the issuer or (2) awards granted in conjunction with selling goods or services to

customers as part of a contract and services from nonemployees. ASU 2018-07 specifies that Topic 718 applies to all share-based payment transactions accounted for under ASC 606. ASU 2018-07 is effective for annual and interim periods beginning after December 15, 2018, with early adoption permitted, but no earlier than the date on which ASC 606 is adopted. 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.

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. In July 2018, the FASB issued ASU 2018-11, Leases (Topic 842): Targeted Improvements, which provides entities with relief from the costs of implementing certain aspects of the new leasing standard, ASU 2016-02. Under the amendments in ASU 2018-11, entities may elect not to restate the comparative periods presented when

F-19

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## Table of Contents

transitioning to ASC 842 (optional transition method) and lessors may elect not to separate lease and non-lease components when certain conditions are met (lessor relief practical expedient). The optional transition method applies to entities that have not yet adopted ASU 2016-02, which 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. The Company's analysis includes, but is not limited to, reviewing existing leases, reviewing other service agreements for embedded leases, establishing policies and procedures, assessing potential disclosures and evaluating the impact of adoption on the Company's consolidated financial statements. The Company expects to recognize a lease liability and related right-of-use asset on its consolidated balance sheets upon adoption of this standard and expects the impact to its consolidated statements of operations and comprehensive loss will not be material.

### Recently Adopted Accounting Standards Updates

In May 2017, the FASB issued 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 was effective for annual and interim periods beginning after December 15, 2017, with early adoption permitted. The Company adopted this standard prospectively effective January 1, 2018. The adoption of this ASU did not have an effect on the Company's consolidated financial statements or related disclosures.

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 was effective for annual and interim periods beginning after December 15, 2017, with early adoption permitted. The Company adopted this standard effective January 1, 2018. Upon adoption of ASU 2016-18, the Company applied the retrospective transition method for each period presented and included approximately \$162,000 of restricted cash in the beginning-of-period and end-of-period cash, cash equivalents and restricted cash balance reflected in the consolidated statements of cash flows for the year ended December 31, 2017. A reconciliation of cash, cash equivalents and restricted cash for each period presented is provided in Note 2 to the consolidated financial statements.

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 was effective for annual and interim periods beginning after December 15, 2017, with early adoption permitted. The Company adopted this standard effective January 1, 2018. The adoption of this ASU did not have an effect on the Company's consolidated financial statements or related disclosures.



In May 2014, the FASB issued ASU 2014-09, Revenue from Contracts with Customers (Topic 606) which amends the guidance for accounting for revenue from contracts with customers. This ASU supersedes the revenue recognition requirements in ASC Topic 605, Revenue Recognition. In 2015 and 2016, the FASB issued additional ASUs related to ASC 606 that delayed the effective date of the guidance and clarified various aspects of the new revenue guidance, including principal versus agent considerations, identifying performance obligations, and licensing, and they include other improvements and practical expedients. The Company adopted this new standard on January 1, 2018 using the full retrospective method. There was no change to the Company's consolidated financial statements as a result of the adoption.

F-20

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Table of Contents

## 3. Inventory

During the third quarter of 2018, the Company began capitalizing inventory costs for COPIKTRA manufactured in preparation for its launch in the United States based on its evaluation of, among other factors, the status of the COPIKTRA New Drug Application (NDA) in the United States and the ability of its third-party suppliers to successfully manufacture commercial quantities of COPIKTRA, which provided the Company with reasonable assurance that the net realizable value of the inventory would be recoverable.

Inventory consists of the following (in thousands):

	December 31, 2018	December 31, 2017
Raw materials	\$ —	\$ —
Work in process	63	—
Finished goods	264	—
Total inventories	\$ 327	\$ —

Costs incurred prior to the quarter-ended September 30, 2018 to manufacture COPIKTRA were expensed as operating expenses as incurred.

## 4. Property and equipment, net

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

	December 31, 2018	December 31, 2017
Leasehold improvements	\$ 146	\$ 2,104
Laboratory equipment	—	908
Furniture and fixtures	1,074	325
Computer equipment	658	279
	1,878	3,616
Less: accumulated depreciation	(509)	(2,755)
Total property and equipment, net	\$ 1,369	\$ 861

During the year ended December 31, 2018, an amendment to the Company's existing office space lease was executed whereby the Company relocated from its previous 15,197 rentable square foot location to an adjacent 27,810 rentable square foot location within the same building. As a result of this amendment, the Company shortened the useful life

of the leasehold improvements related to the original location and depreciated this balance through the date which it vacated the original space. Upon vacating the original 15,197 rentable office space, the Company disposed of the leasehold improvements related to this location. No gain or loss from the disposal of leasehold improvements was recognized during the year ended December 31, 2018.

The Company recorded approximately \$1.0 million, \$0.6 million, and \$0.7 million in depreciation expense for the years ended December 31, 2018, 2017 and 2016, respectively.

Table of Contents

## 5. Intangible assets

The Company's intangible assets consist of the following (in thousands):

	December 31, 2018	Estimated useful life
Acquired and in-licensed rights	\$ 22,000	14 years
Less: accumulated amortization	(423)	
Total intangible assets, net	\$ 21,577	

Acquired and in-licensed rights as of December 31, 2018, consist of a \$22.0 million milestone payment which became payable upon the FDA marketing approval on September 24, 2018 pursuant to the amended and restated license agreement with Infinity. The Company made a milestone payment of \$22.0 million to Infinity in November 2018.

The Company recorded approximately \$0.4 million in amortization expense related to finite-lived intangible assets during the year ended December 31, 2018 using the straight-line methodology. Estimated future amortization expense for finite-lived intangible assets as of December 31, 2018 is approximately \$1.6 million per year thereafter.

## 6. Accrued expenses

Accrued expenses consist of the following (in thousands):

	December 31, 2018	December 31, 2017
Compensation and related benefits	8,749	2,622
Contract research organization costs	6,682	3,774
Commercialization costs	1,979	131
Interest	1,786	108
Consulting fees	494	448
Professional fees	482	617
Other	936	242
Total accrued expenses	\$ 21,108	\$ 7,942

## 7. 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 Capital, Inc. (Hercules). 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 an aggregate of \$15.0 million were drawn down during the year ended December 31, 2017. 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, while the remaining proceeds were used for ongoing research and

development programs and for general corporate purposes.

The Company executed several amendments to the Original Loan Agreement prior to March 6, 2018 which increased the borrowing limit under the Original Loan Agreement from up to \$25.0 million to up to \$50.0 million (the Term Loan), resulting in \$35.0 million of borrowing capacity remaining under the Amended Loan Agreement. The Company drew down an additional \$10.0 million in June 2018. The remaining \$25.0 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 the Company's New Drug

F-22

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## Table of Contents

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 Amended 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 Amended Loan Agreement, including put and call features. The Company determined that all features of the Amended 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 consolidated financial statements. The Company reassesses the features on a quarterly basis to determine if they require separate accounting. There have been no changes to the Company's original assessment through December 31, 2018.

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The future principal payments under the Amended Loan Agreement are as follows as of December 31, 2018 (in thousands):

2019	\$ 5,930
2020	19,070
Total principal payments	\$ 25,000

F-23

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Table of Contents

## 8. Product revenue reserves and allowances

As of December 31, 2018, the Company's sole source of product revenue has been from sales of COPIKTRA in the United States, which it began shipping to customers on September 25, 2018. The following table summarizes activity in each of the product revenue allowance and reserve categories for the year ended December 31, 2018 (in thousands):

	Trade discounts and allowances	Third-Party Payer chargebacks, discounts and fees	Government rebates and other incentives	Returns	Total
Beginning balance at December 31, 2017	\$ —	\$ —	\$ —	\$ —	\$ —
Provision related to sales in the current year	69	120	157	2	348
Adjustments related to prior period sales	—	—	—	—	—
Credits and payments made	(40)	(32)	—	—	(72)
Ending balance at December 31, 2018	\$ 29	\$ 88	\$ 157	\$ 2	\$ 276

Trade discounts and Third-Party Payer chargebacks and discounts are recorded as a reduction to accounts receivable, net on the consolidated balance sheets. Trade allowances and Third-Party Payer fees, government rebates, other incentives and returns are recorded as a component of accrued expenses on the consolidated balance sheets.

## 9. Common stock

As of December 31, 2018 and 2017, 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,	
	2018	2017
Shares reserved under equity compensation plans	15,572	8,264
Shares reserved for inducement grants	6,381	3,616
Shares reserved for Convertible Senior Notes	20,937	—
Total shares reserved	42,890	11,880

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

On March 30, 2017, 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, 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, 2018, the Company sold 11,518,354 shares under the 2017 ATM Program for net proceeds of approximately \$47.3 million (after deducting commissions and other offering



expenses).

F-24

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Table of Contents

## Equity offering

On May 16, 2018, the Company entered into an underwriting agreement with Cantor relating to the underwritten offering of 7,777,778 shares (the Shares) of the Company's common stock (the Underwriting Agreement). Cantor agreed to purchase the Shares pursuant to the Underwriting Agreement at a price of \$4.31 per share. In addition, the Company granted Cantor an option to purchase, at the public offering price less any underwriting discounts and commissions, an additional 1,166,666 shares of the Company's common stock, exercisable for 30 days from the date of the prospectus supplement. The option was exercised by Cantor in full on May 23, 2018. The aggregate proceeds from Cantor, net of underwriting discounts and offering costs, were approximately \$38.3 million.

On June 14, 2018, the Company entered into a purchase agreement with Consonance Capital Master Account L.P. and P Consonance Opportunities Ltd. (collectively, Consonance) relating to the registered offering of 7,166,666 shares of its common stock at a price of \$6.00 per share. The aggregate proceeds from Consonance, net of offering costs, were approximately \$42.9 million.

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.

## 10. 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,		
	2018	2017	2016
Research and development	\$ 2,043	\$ 1,381	\$ 1,073
Selling, general and administrative	4,628	3,652	5,145
Total stock-based compensation expense	\$ 6,671	\$ 5,033	\$ 6,218

All of the \$6.7 million and \$5.0 million of stock-based compensation expense recorded during the years ended December 31, 2018 and 2017, respectively, 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.

The Company has awards outstanding under two equity compensation plans, the Amended and Restated 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 included an “evergreen provision” that allowed for an annual increase in the number of shares of common stock available for issuance under the 2012 Plan. The annual increase was added on the first day of each year from 2013 through 2018 and was equal to the lesser of 1,285,714 shares of common stock and 4.0% of the number of shares of common

F-25

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Table of Contents

stock outstanding, or a lesser amount as determined by the board of directors. On each of January 1, 2018, January 1, 2017 and January 1, 2016, the number of shares available for issuance under the 2012 Plan increased by 1,285,714 under this provision. On December 18, 2018, the shareholders of the Company approved the Amended and Restated 2012 Incentive Plan which increased the maximum number of shares available for issuance under the 2012 Plan to 16,628,425 and eliminated the evergreen 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, 2018, under the 2012 Plan, the Company has granted stock options for 12,099,594 shares of common stock, of which 3,683,349 have been forfeited and 364,958 have been exercised, and restricted stock units for 1,195,918 shares of common stock, of which 219,351 have been forfeited and 759,817 have vested. 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, and RSUs in respect of up to an aggregate of 750,000 shares of common stock to new or prospective employees as inducement to enter into employment with the Company. 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. In June and December 2018, the Board of Directors authorized and reserved 1,700,000 and 1,250,000 additional shares of common stock under this program, respectively. The program is governed by the terms of the 2012 Plan but shares issued pursuant to the program are not issued under the 2012 Plan. As of December 31, 2018, the Company had granted options for 5,447,841 shares of common stock under the program, of which 721,082 have been forfeited and 323,750 have been exercised, and restricted stock units for 90,000 shares, of which none have been forfeited nor vested. As of December 31, 2018, 1,888,241 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, 2018 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, 2017	8,719,978	\$ 5.19	7.9	\$ 6,150
Granted	5,539,232	\$ 5.33		
Exercised	(412,851)	\$ 1.96		
Forfeited/cancelled	(1,323,492)	\$ 4.55		
Outstanding at December 31, 2018	12,522,867	\$ 5.42	7.8	\$ 6,909
Vested at December 31, 2018	6,107,429	\$ 6.05	6.5	\$ 4,469

Vested and expected to vest at December 31, 2018(1)	12,084,867	\$ 5.46	7.8	\$ 6,811
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(1) This represents the number of vested options as of December 31, 2018, plus the number of unvested options expected to vest as of December 31, 2018.

F-26

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Table of Contents

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

	Year ended December 31,		
	2018	2017	2016
Risk-free interest rate	2.65 %	2.02 %	1.48 %
Volatility	81 %	78 %	75 %
Dividend yield	—	—	—
Expected term (years)	5.8	5.8	5.9

The Company recorded stock based compensation expense associated with employee stock options of \$5.6 million, \$4.5 million, and \$6.1 million, for the years ended December 31, 2018, 2017, and 2016, respectively. The weighted average grant date fair value of options granted in the years ended December 31, 2018, 2017, and 2016 was \$3.72, \$1.83, and \$0.99 per share, respectively. The fair value of options that vested during the years ended December 31, 2018, 2017, and 2016 was \$4.7 million, \$4.8 million, and \$6.9 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, 2018 and 2017 was \$1.8 million and \$1.1 million, respectively.

During the first quarter of 2018, the Company granted stock options to purchase a total of 582,500 shares of common stock to certain executives that vest only upon the achievement of specified performance conditions. The Company determined that two of the performance conditions had been achieved as of December 31, 2018. The Company has recognized approximately \$0.7 million of stock-based compensation expense during the year ended December 31, 2018 related to awards that vest upon the achievement of performance conditions. As of December 31, 2018, a total of 438,000 options remain unvested which are related to the future achievement of performance conditions.

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 \$0.2 million 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 \$0.4 million 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, 2018, there was \$17.5 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 3.7 years.

#### Restricted Stock Units

The Company awards RSUs to employees under its 2012 Incentive Plan and Inducement Award Program. Each RSU entitles the holder to receive one share of the Company's common stock when the RSU vests. The RSUs generally vest in either (i) four substantially equal installments on each of the first four anniversaries of the vesting commencement date, or (ii) 100 percent on the first anniversary of the vesting commencement date, subject to the employee's

continued employment with, or service to, the Company on such vesting date. Compensation expense is recognized on a straight-line basis.

F-27

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Table of Contents

A summary of RSU activity during the year ended December 31, 2018 is as follows:

	Shares	Weighted-average grant date fair value per share
Outstanding at December 31, 2017	—	\$ —
Granted	376,000	\$ 5.28
Vested	—	\$ —
Forfeited	(69,250)	\$ 5.45
Outstanding at December 31, 2018	306,750	\$ 5.24

The Company recorded stock based compensation expense associated with employee RSUs of \$0.4 million, less than \$0.1 million, and \$0.1 million, for the years ended December 31, 2018, 2017, and 2016, respectively. No RSUs were granted during the years ended December 31, 2017 and 2016. The total fair value of restricted stock units vested during the years ended December 31, 2018, 2017, and 2016 was insignificant.

At December 31, 2018, there was \$1.2 million of total unrecognized compensation cost related to unvested RSUs and the Company expects to recognize this cost over a remaining weighted-average period of 1.87 years.

#### 11. Convertible Senior Notes

On October 17, 2018, the Company closed a registered direct public offering of \$150.0 million aggregate principal amount of the Company's 5.00% Convertible Senior Notes due 2048 (the Notes), for net proceeds of approximately \$145.3 million. The Notes are governed by the terms of a base indenture for senior debt securities (the Base Indenture), as supplemented by the first supplemental indenture thereto (the Supplemental Indenture and together with the Base Indenture, the Indenture), each dated October 17, 2018, by and between the Company and Wilmington Trust, National Association, as trustee. The Notes are senior unsecured obligations of the Company and bear interest at a rate of 5.00% per annum, payable semi-annually in arrears on May 1 and November 1 of each year, beginning on May 1, 2019. The Notes will mature on November 1, 2048, unless earlier repurchased, redeemed or converted in accordance with their terms.

The Notes are convertible into shares of the Company's common stock, par value \$0.0001 per share, together, if applicable, with cash in lieu of any fractional share, at an initial conversion rate of 139.5771 shares of common stock per \$1,000 principal amount of the Notes, which corresponds to an initial conversion price of approximately \$7.16 per share of common stock and represents a conversion premium of approximately 15.0% above the last reported sale price of the common stock of \$6.23 per share on October 11, 2018. Upon conversion, converting noteholders will be entitled to receive accrued interest on their converted Notes. To the extent the Company has insufficient authorized but unissued shares to settle conversions in shares of common stock, the Company would be required to settle the deficiency in cash.

The Company will have the right, exercisable at its option, to cause all Notes then outstanding to be converted automatically if the "Daily VWAP" (as defined in the Indenture) per share of the Company's common stock equals or exceeds 130% of the conversion price on each of at least 20 VWAP Trading Days (as defined in the Indenture), whether or not consecutive, during any 30 consecutive VWAP Trading Day period commencing on or after the date the Company first issued the Notes.



The conversion rate is subject to adjustment from time to time upon the occurrence of certain events, including, but not limited to, the issuance of stock dividends and payment of cash dividends, but will not be adjusted for any accrued and unpaid interest.

Prior to November 1, 2022, the Company will not have the right to redeem the Notes. On or after November 1, 2022, the Company may elect to redeem the Notes, in whole or in part, at a cash redemption price equal to the principal amount of the Notes to be redeemed, plus accrued and unpaid interest, if any.

Unless the Company has previously called all outstanding Notes for redemption, the Notes will be subject to repurchase by the Company at the holders' option on each of November 1, 2023, November 1, 2028,

F-28

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Table of Contents

November 1, 2033, November 1, 2038 and November 1, 2043 (or, if any such date is not a business day, on the next business day) at a cash repurchase price equal to the principal amount of the Notes to be repurchased, plus accrued and unpaid interest, if any.

If a “Fundamental Change” (as defined in the Indenture) occurs at any time, subject to certain conditions, holders may require the Company to purchase all or any portion of their Notes at a purchase price equal to 100% of the principal amount of the Notes to be purchased, plus accrued and unpaid interest. If a “Fundamental Change” occurs on or before November 1, 2022 and a holder elects to convert its Notes in connection with such change, such holder may be entitled to an increase in the conversion rate in certain circumstances as set forth in the Indenture.

The Notes are the Company’s senior, unsecured obligations and will be senior in right of payment to the Company’s future indebtedness that is expressly subordinated in right of payment to the Notes; equal in right of payment with the Company’s existing and future indebtedness that is not so subordinated, and effectively subordinated to the Company’s existing and future secured indebtedness, to the extent of the value of the collateral securing such indebtedness. The Notes are structurally subordinated to all existing and future indebtedness and other liabilities, including trade payables, and (to the extent the Company is not a holder thereof) preferred equity, if any, of the Company’s subsidiaries.

The Indenture includes customary covenants and set forth certain events of default after which the Notes may be declared immediately due and payable and set forth certain types of bankruptcy or insolvency events of default involving the Company or certain of its subsidiaries after which the Notes become automatically due and payable

The Company assessed all terms and features of the Notes in order to identify any potential embedded features that would require bifurcation. As part of this analysis, the Company assessed the economic characteristics and risks of the Notes, including the conversion, put and call features. Per the terms of the Indenture, upon conversion of the Notes, a portion of the principal may be settled in cash until the date upon which the Company’s stockholders approve an increase in the number of authorized shares of common stock, or the Authorized Share Effective Date, as defined. In consideration of this provision, the Company concluded the conversion feature required bifurcation as a derivative. The fair value of the conversion feature derivative was determined based on the difference between the fair value of the Notes with the conversion option and the fair value of the Notes without the conversion option. The Company determined that the fair value of the derivative upon issuance of the Notes was \$51.5 million and recorded this amount as a derivative liability and the offsetting amount as a debt discount as a reduction to the carrying value of the Notes on the closing date, or October 17, 2018.

On December 18, 2018, the Authorized Share Effective Date was achieved as the Company’s stockholders approved an increase in the number of authorized shares of Common Stock. Following this approval, no portion of the Notes are settleable in cash upon conversion. As such, the Company determined that the conversion feature no longer met the definition of a derivative following the increase in the number of authorized shares of common stock. As of December 18, 2018, the Company determined the fair value of the conversion feature was \$25.9 million. The Company recorded the change in the fair value of the conversion feature for the period from October 17, 2018 to December 18, 2018 of \$25.6 million as other income on the consolidated statements of operations and comprehensive

loss. As of December 18, 2018, the fair value of the conversion option was reclassified to additional paid-in capital on the consolidated balance sheets as it qualified for a scope exception from derivative accounting. Accordingly, the conversion feature will no longer be measured at fair value on the Company's financial statements.

The Company determined that all other features of the Notes 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 consolidated financial statements. The Company reassesses the features on a quarterly basis to determine if they require separate accounting. There have been no changes to the Company's original assessment through December 31, 2018.

The Company determined that the expected life of the Notes was equal to the period through November 1, 2023 as this represents the point at which the Notes are initially subject to repurchase by the Company at the option of the holders. Accordingly, the total debt discount of \$56.3 million, inclusive of the fair value of the embedded conversion feature derivative at issuance, is being amortized using the effective interest method through November

Table of Contents

1, 2023. For the year ended December 31, 2018, the Company recognized \$3.1 million of interest expense related to the Notes.

## 12. Net Loss per Share

ASC 260 "Earnings Per Share" requires the Company to calculate its net loss per share based on basic and diluted net loss per share, as defined. Basic EPS excludes dilution and is computed by dividing net loss by the weighted average number of shares outstanding for the period. For the year ended December 31, 2018, the dilutive effect of the outstanding Notes issued by the Company is reflected in diluted EPS using the if-converted method. For the years ended December 31, 2017 and 2016 periods of net loss, basic and diluted EPS are the same as the assumed exercise of stock options, warrants and restricted stock units are anti-dilutive.

The computation of basic and diluted net income (loss) per share attributable to common stockholders consists of the following:

	Year Ended December 31,		
	2018	2017	2016
Net loss	(72,434)	(67,802)	(36,440)
Less: Other income	(25,556)	—	—
Add: Interest expense	3,071	—	—
Adjusted diluted net loss	(94,919)	(67,802)	(36,440)
Weighted average shares outstanding	64,962	38,422	36,988
Add: Dilutive effect of the Notes	4,359	—	—
Weighted average diluted shares outstanding	69,321	38,422	36,988
Net loss per share - basic	(1.12)	(1.76)	(0.99)
Net loss per share - diluted	(1.37)	(1.76)	(0.99)

In calculating the effect of the Notes on diluted net loss per share, the change in fair value of the bifurcated derivative of \$25.6 million is subtracted while the interest expense of \$3.1 million is added to the Company's net loss. As of December 31, 2018, upon conversion of all outstanding Notes, the Company would be required to issue 20,936,548 shares. Under the "if-converted" method, convertible instruments are assumed to have been converted as of the beginning of the period or when issued, if later. Accordingly, the weighted average number of potentially issuable shares upon conversion of the Notes was determined by weighting the number of shares potentially issuable as of December 31, 2018, 20,936,548 shares, over the total number of days the Notes were outstanding for the period, 76 days, to calculate an additional 4,359,391 shares to be added to the denominator.

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,		
	2018	2017	2016
Outstanding stock options	12,522,867	8,719,978	5,848,470

Outstanding warrants	—	—	142,857
Outstanding restricted stock units	306,750	—	—
Total potentially dilutive securities	12,829,617	8,719,978	5,991,327

### 13. Income Taxes

As of December 31, 2018, the Company had federal and state net operating loss carryforwards of approximately \$325.2 million and \$325.0 million, respectively, which are available to reduce future taxable income. The Company also had federal and state tax credits of \$18.1 million and \$2.0 million, respectively, which may be

F-30

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Table of Contents

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.

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

	December 31,			
	2018	%	2017	%
Income tax benefit using U.S. federal statutory rate	21.00	%	34.00	%
State tax benefit, net of federal benefit	6.38	%	5.00	%
Research and development tax credits	5.61	%	7.04	%
Permanent items	(0.65)	%	(1.24)	%
Effect of U.S. Tax Cuts and Jobs Act	—	%	(45.19)	%
Change in the valuation allowance	(31.82)	%	0.19	%
Other	(0.52)	%	0.20	%
	—	%	—	%

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

	December 31,	
	2018	2017
Deferred tax assets:		
Net operating loss carryforwards	\$ 88,829	\$ 64,677
Capitalized research and development	2,545	2,780
Research and development credits	19,725	15,406
Stock-based compensation	3,756	2,560
Other	543	379
Total deferred tax assets	115,398	85,802
Deferred tax liabilities:		
Derivative liability	(13,617)	—
Total deferred tax liabilities	(13,617)	—
Net deferred tax asset	101,781	85,802
Valuation allowance	(101,781)	(85,802)
Net deferred tax asset	\$ —	\$ —

The Company has recorded a valuation allowance against its deferred tax assets at December 31, 2018 and 2017 because the Company's management believes that it is more likely than not that these assets will not be fully realized. The increase in the valuation allowance of approximately \$16.0 million in the year ended December 31, 2018 primarily relates to the generation of net operating losses and research and development credits.

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, 2018, 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

F-31

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Table of Contents

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.

During 2017, the Company recorded tax charges for the impact of the Tax Act effects using the current available information and technical guidance on the interpretations of the Tax Act. As permitted by SEC Staff Accounting Bulletin 118, Income Tax Accounting Implications of the Tax Cuts and Jobs Act, the Company recorded provisional estimates and have subsequently finalized our accounting analysis based on the guidance, interpretations, and data available as of December 31, 2018. Adjustments made in the fourth quarter of 2018 upon finalization of our accounting analysis were not material to our consolidated financial statements.

## 14. 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 was scheduled to expire on September 30, 2019. Effective February 15, 2018, the Company amended its lease agreement to relocate within the facility to another location consisting of 27,810 square feet of office space (the Amended Lease Agreement). The Amended Lease Agreement extends the expiration date of the lease from September 2019 through May 2025. Pursuant to the Amended Lease Agreement, the initial annual base rent amount is approximately \$660,000, which increases during the lease term to \$1.1 million for the last twelve-month period. The deferred rent obligation is 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. In conjunction with the execution of the Amended Lease Agreement, the Company increased its security deposit by increasing its existing letter of credit to approximately \$403,000, which was reduced to \$241,000 in October 2018. The amount is included in restricted cash on the consolidated balance sheets as of December 31, 2018.

The minimum aggregate future lease commitments as of December 31, 2018 are as follows (in thousands):

2019	\$ 716
2020	971
2021	1,020
2022	1,041
2023	1,062
Thereafter	1,538
Total	\$ 6,348

The Company recorded rent expense of approximately \$0.8 million, \$0.4 million and \$0.4 million for the years ended December 31, 2018, 2017 and 2016, 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.

## 15. License and collaboration agreements



Infinity

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

F-32

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## Table of Contents

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 maintained a portion of the financial responsibility for the shutdown of certain other clinical studies. 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 was required to make the following payments to Infinity in cash or, at the Company's election, in whole or in part, in shares of the Company's common stock: (i) \$6.0 million upon the completion of the DUO study if the results of the DUO study met certain pre-specified criteria, which was paid in cash by the Company to Infinity in October 2017 and recorded as research and development expense in the consolidated statements of operations and comprehensive loss, 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, which was paid in cash by the Company to Infinity in November 2018 and recorded as an intangible asset in the consolidated balance sheets.

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 the Company 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 Company 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.

During the year ended December 31, 2018, the Company paid Infinity \$0.1 million related to the Infinity, MICL, and Purdue royalty payments, which are included in costs of revenue within the consolidated statements of operation. There were no royalties paid to Infinity during the years ended December 31, 2017 and 2016.

Pfizer Inc. (Pfizer)

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,

F-33

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## Table of Contents

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 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.

### Yakult Honsha Co., Ltd. (Yakult)

On June 5, 2018, the Company entered into a license and collaboration agreement (the Yakult Agreement) with Yakult, under which the Company granted exclusive rights to Yakult to develop and commercialize products containing duvelisib in Japan for the treatment, prevention, palliation or diagnosis of all oncology indications in humans or animals.

Under the terms of the Yakult Agreement, Yakult received an exclusive right to develop and commercialize products containing duvelisib in Japan under mutually agreed upon development and commercialization plans at its own cost and expense. Yakult also received certain limited manufacturing rights in the event that the Company is unable to manufacture or supply sufficient quantities of duvelisib or products containing duvelisib to Yakult during the term of the Yakult Agreement. The Company retained all rights to duvelisib outside of Japan.

Yakult paid the Company an upfront, non-refundable payment of \$10.0 million in June 2018. The Company is also entitled to receive aggregate payments of up to \$90.0 million if certain development, regulatory and commercial milestones are successfully achieved. Yakult is obligated to pay the Company a double-digit royalty on net sales of products containing duvelisib in Japan, subject to reduction in certain circumstances, and to fund certain global development costs related to worldwide clinical trials conducted by the Company in which Yakult has opted to participate (Global Clinical Trials) on a pro-rata basis.

Unless earlier terminated by either party, the Yakult Agreement will expire upon the fulfillment of Yakult's royalty obligations to the Company for the sale of any products containing duvelisib in Japan, which royalty obligations expire, on a product-by-product basis, upon the last to occur of (a) expiration of valid claims covering such product, (b) expiration of regulatory exclusivity for such product or (c) 10 years from first commercial sale of such product. Yakult may terminate the Yakult Agreement in its entirety at any time with 180 days' written notice. Either party may terminate the Yakult Agreement in its entirety with 60 days' written notice for the other party's material breach if such party fails to cure the breach. The Company may terminate the Yakult Agreement if (i) Yakult fails to use commercially reasonable efforts to develop and commercialize products containing duvelisib in Japan or (ii) Yakult challenges any patent licensed by the Company to Yakult under the Yakult Agreement. Either party may terminate the Yakult Agreement in its entirety upon certain insolvency events involving the other party.

The Company first assessed the Yakult Agreement under ASC 808 to determine whether the Yakult Agreement (or part of the Yakult Agreement) represents a collaborative arrangement based on the risks and rewards and activities of the parties pursuant to the Yakult Agreement. The Company accounts for collaborative arrangements (or elements

within the contract that are deemed part of a collaborative arrangement), which represent a collaborative relationship and not a customer relationship, outside the scope of ASC 606. For a component of the Yakult Agreement, the Company concluded that both the Company and Yakult are exposed to significant risks while developing duvelisib and ultimately would share in the reward upon successful commercialization of duvelisib. The Company then considered each remaining component in the Yakult Agreement to determine if ASC 606 should be

F-34

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## Table of Contents

applied to those components. Generally, the components in the Yakult Agreement fall under one of two potential research and development activities: (i) the parties' joint participation in Global Clinical Trials and (ii) the territory-specific development of duvelisib.

For the parties' participation in the Global Clinical Trials, the Company concluded that the research and development activities and payments related to such activities are not within the scope of ASC 606 as Yakult is not a customer of the Company with regards to these activities in the context of the Yakult Agreement. As such, costs incurred to execute the Global Clinical Trials will be recorded as research and development expense and payments received from Yakult related to such will be recorded as a reduction of research and development expense.

For Territory-specific activities, the Company concluded that Yakult is a customer with regard to this component in the context of the Yakult Agreement. As such, the Territory-specific component and all related payments are within the scope of ASC 606.

The Company determined that there were two material promises associated with the territory-specific activities: (i) an exclusive license to develop and commercialize duvelisib in the territory and (ii) the initial technology transfer. The Company determined that the exclusive license and initial technology transfer were not distinct from another, as the license has limited value without the initial technology. Therefore, the exclusive license and initial technology transfer are combined as a single performance obligation. The Company evaluated the option rights for manufacturing and supply services to determine whether they represent material rights to Yakult and concluded that the options were not issued at a significant and incremental discount and therefore do not represent material rights. As such, they are not performance obligations at the outset of the arrangement. Based on this assessment, the Company concluded one performance obligation exists at the outset of the Yakult Agreement: the exclusive license combined with the initial technology transfer.

The Company determined that the upfront payment of \$10.0 million constitutes the transaction price as of the outset of the Yakult Agreement. Future potential milestone payments were fully constrained as the risk of significant revenue reversal related to these amounts has not yet been resolved. The achievement of the future potential milestones is not within the Company's control and is subject to certain research and development success or regulatory approvals and therefore carry significant uncertainty. The Company will reevaluate the likelihood of achieving future milestones at the end of each reporting period. As all performance obligations have been satisfied, if the risk of significant revenue reversal is resolved, any future milestone revenue from the arrangement will be added to the transaction price (and thereby recognized as revenue) in the period the risk is relieved.

The Company satisfied the performance obligation upon delivery of the license and initial technology transfer and recognized the upfront payment of \$10.0 million as license revenue during year ended December 31, 2018.

### CSPC Pharmaceutical Group Limited (CSPC)

On July 26, 2018, the Company and CSPC entered into an Exclusivity Agreement which granted CSPC the exclusive right to negotiate a licensing agreement with the Company for duvelisib in China. CSPC paid the Company a non-refundable exclusivity fee of \$5.0 million in August 2018 (Exclusivity Fee) which was creditable against any payments agreed to under the terms of a potential definitive license agreement.

Subsequently, on September 25, 2018, the Company entered into a license and collaboration agreement with CSPC (the CSPC Agreement), under which the Company granted exclusive rights to CSPC to develop and commercialize products containing duvelisib in the People's Republic of China (China), Hong Kong, Macau and Taiwan (collectively,

the CSPC Territory) for the treatment, prevention, palliation or diagnosis of all oncology indications in humans.

Under the terms of the CSPC Agreement, CSPC received an exclusive right to develop and commercialize products containing duvelisib in the CSPC Territory under mutually agreed upon development and commercialization plans at its own cost and expense. CSPC also received certain limited manufacturing rights in the

F-35

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## Table of Contents

event that the Company is unable to manufacture or supply sufficient quantities of duvelisib or products containing duvelisib to CSPC during the term of the CSPC Agreement. The Company retained all rights to duvelisib outside of the CSPC Territory.

CSPC paid the Company an aggregate upfront, non-refundable payment of \$15.0 million, less the previously paid \$5.0 million Exclusivity Fee. The Company is also entitled to receive aggregate payments of up to \$160.0 million if certain development, regulatory and commercial milestones are successfully achieved. CSPC is obligated to pay the Company a double-digit royalty on net sales of products containing duvelisib in the CSPC Territory, subject to reduction in certain circumstances, and to fund certain global development costs related to worldwide clinical trials conducted by the Company in which CSPC has opted to participate (Global Clinical Trials) on a pro-rata basis.

Unless earlier terminated by either party, the CSPC Agreement will expire upon the fulfillment of CSPC's royalty obligations to the Company for the sale of any products containing duvelisib in the CSPC Territory, which royalty obligations expire, on a product-by-product basis, upon the last to occur of (a) expiration of valid claims covering such product, (b) expiration of regulatory exclusivity for such product or (c) 10 years from first commercial sale of such product. CSPC may terminate the CSPC Agreement in its entirety at any time with 180 days' written notice. Either party may terminate the CSPC Agreement in its entirety with 60 days' written notice for the other party's material breach if such party fails to cure the breach. The Company may terminate the CSPC Agreement if (i) CSPC fails to use commercially reasonable efforts to develop and commercialize products containing duvelisib in the CSPC Territory or (ii) CSPC challenges any patent licensed by the Company to CSPC under the CSPC Agreement. Either party may terminate the CSPC Agreement in its entirety upon certain insolvency events involving the other party.

The Company first assessed the CSPC Agreement under ASC 808 to determine whether the CSPC Agreement (or part of the CSPC Agreement) represents a collaborative arrangement based on the risks and rewards and activities of the parties pursuant to the CSPC Agreement. The Company accounts for collaborative arrangements (or elements within the contract that are deemed part of a collaborative arrangement), which represent a collaborative relationship and not a customer relationship, outside the scope of ASC 606. For a component of the CSPC Agreement, the Company concluded that both the Company and CSPC are exposed to significant risks while developing duvelisib and ultimately would share in the reward upon successful commercialization of duvelisib. The Company then considered each remaining component in the CSPC Agreement to determine if ASC 606 should be applied to those components. Generally, the components in the CSPC Agreement fall under one of two potential research and development activities: (i) the parties' joint participation in Global Clinical Trials and (ii) the territory-specific development of duvelisib.

For the parties' participation in the Global Clinical Trials, the Company concluded that the research and development activities and payments related to such activities are not within the scope of ASC 606 as CSPC is not a customer of the Company with regards to these activities in the context of the CSPC Agreement. As such, costs incurred to execute the Global Clinical Trials will be recorded as research and development expense and payments received from CSPC related to such will be recorded as a reduction of research and development expense.

For CSPC Territory-specific activities, the Company concluded that CSPC is a customer with regard to this component in the context of the CSPC Agreement. As such, the CSPC Territory-specific component and all related payments are within the scope of ASC 606.

The Company determined that there were two material promises associated with the territory-specific activities: (i) an exclusive license to develop and commercialize duvelisib in the territory and (ii) the initial technology transfer. The Company determined that the exclusive license and initial technology transfer were not distinct from another, as the license has limited value without the initial technology. Therefore, the exclusive license and initial technology transfer are combined as a single performance obligation. The Company evaluated the option rights for manufacturing and



supply services to determine whether they represent material rights to CSPC and concluded that the options were not issued at a significant and incremental discount and therefore do not represent material rights. As such, they are not performance obligations at the outset of the arrangement. Based on this

F-36

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Table of Contents

assessment, the Company concluded one performance obligation exists at the outset of the CSPC Agreement: the exclusive license combined with the initial technology transfer.

The Company determined that the upfront payment of \$15.0 million constitutes the transaction price as of the outset of the CSPC Agreement. Future potential milestone payments were fully constrained as the risk of significant revenue reversal related to these amounts has not yet been resolved. The achievement of the future potential milestones is not within the Company's control and is subject to certain research and development success or regulatory approvals and therefore carry significant uncertainty. The Company will reevaluate the likelihood of achieving future milestones at the end of each reporting period. As all performance obligations have been satisfied, if the risk of significant revenue reversal is resolved, any future milestone revenue from the arrangement will be added to the transaction price (and thereby recognized as revenue) in the period the risk is relieved.

The Company satisfied the performance obligation upon delivery of the license and initial technology transfer and recognized the upfront payment of \$15.0 million as license revenue during the year ended December 31, 2018.

16. 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 contributions to the 401(k) Plan of approximately \$0.8 million, \$0.3 million, and \$0.2 million for the years ended December 31, 2018, 2017, and 2016, respectively.

Table of Contents

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

	First Quarter Ended	Second Quarter Ended	Third Quarter Ended	Fourth Quarter Ended
	March 31, 2018	June 30, 2018	September 30, 2018	December 31, 2018
Revenue:				
Product revenue, net	\$ —	\$ —	\$ 508	\$ 1,210
License revenue	—	10,000	15,000	—
Total revenue	—	10,000	15,508	1,210
Operating expenses:				
Costs of revenues, excluding amortization of acquired intangible assets	\$ —	\$ —	\$ 49	\$ 116
Research and development	10,934	12,381	11,571	8,762
Selling, general and administrative	9,827	15,813	25,426	26,199
Amortization of acquired intangible assets	—	—	31	392
Total operating expenses	20,761	28,194	37,077	35,469
Loss from operations	(20,761)	(18,194)	(21,569)	(34,259)
Other income	—	—	—	25,556
Interest income	191	343	763	1,306
Interest expense	(480)	(516)	(862)	(3,952)
Net loss	\$ (21,050)	\$ (18,367)	\$ (21,668)	\$ (11,349)
Net loss per share —basic	\$ (0.41)	\$ (0.30)	\$ (0.29)	\$ (0.15)
Net loss per share —diluted	\$ (0.41)	\$ (0.30)	\$ (0.29)	\$ (0.37)
Weighted-average number of common shares used in net loss per share —basic and diluted				
Net loss per share —basic	50,835	(a) 61,256	(a)(b) 73,644	73,766
Net loss per share —diluted	50,835	(a) 61,256	(a)(b) 73,644	91,061 (c)

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- (a) In the first and second quarters of 2018, the Company sold 167,065 and 6,314,410 shares of its common stock under the Company's at-the-market equity offering program, which resulted in net proceeds of \$0.6 million and \$23.7 million, respectively.
- (b) In the second quarter of 2018, the Company closed underwritten offerings in which it sold 8,944,444 shares and 7,166,666 shares of its common stock at a price of \$4.31 per share and \$6.00 per share, respectively, for aggregate proceeds, net of underwriting discounts and offering costs, of \$38.3 million and \$42.9 million, respectively.
- (c) In the fourth quarter of 2018, utilizing the "if-converted" method, the Company's Notes are assumed to have been converted as of the issuance date. Accordingly, the weighted average number of potentially issuable shares upon conversion of the Notes was determined by weighting the number of shares issuable upon conversion at December 31, 2018, or 20,936,548, over the total days outstanding, 76 days, to calculate an additional 17,295,409 shares to

be added to the weighted-average number of shares.

F-38

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Table of Contents

	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
Selling, 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 (d)	42,027 (d)(e)

(d) 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.

(e) 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.

## 18. 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.