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

FORM 10-K

(Mark One)

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

For the fiscal year ended December 31, 2019

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 (State or other jurisdiction of incorporation or organization)	**27-3269467** (I.R.S. Employer Identification No.)
117 Kendrick Street, Suite 500 Needham, Massachusetts (Address of principal executive offices)	**02494** (Zip Code)

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

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.0001 par value	VSTM	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 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", "smaller reporting company" and "emerging growth 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 28, 2019 was $110,799,682.

The number of shares outstanding of the registrant's common stock as of March 11, 2020 was 148,410,387.

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 19, 2020 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, 2019.

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FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements related to present facts or current conditions or historical facts, contained in this Annual Report on Form 10-K, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. Such statements relate to, among other things, the development and activity of our lead product, COPIKTRA and our phosphoinositide 3-kinase (PI3K), focal adhesion kinase (FAK) and rapidly accelerated fibrosarcoma (RAF)/ mitogen-activated protein kinase (MEK) 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 similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

Forward-looking statements are not guarantees of future performance and our actual results could differ materially from the results discussed in the forward-looking statements 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 may not realize the operational efficiencies and cost savings from restructuring; that we, Sanofi, CSPC Pharmaceutical Group Limited, Yakult Honsha Co. Ltd., Chugai Pharmaceutical Co., Ltd, or Infinity Pharmaceuticals, Inc. will fail to fully perform under the 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, 2019, 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.

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. Our marketed product, COPIKTRA® (duvelisib) capsules, and most advanced product candidates, defactinib and CH5126766 also referred to as VS-6766, utilize a multi-faceted approach designed to treat cancers originating either in the blood or major organ systems. We are currently developing duvelisib and our product candidates in both preclinical and clinical studies as potential therapies for certain cancers, including leukemia, lymphoma, lung cancer, mesothelioma, ovarian cancer, head and neck cancer, colorectal 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, other pathway inhibitors or other current and emerging standard of care treatments in aggressive cancers that do not adequately respond to 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 and T-cells. PI3K signaling may lead to the proliferation of malignant B-cells and T-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 for treatment of patients with peripheral T-cell lymphoma (PTCL). The development of duvelisib in PTCL has been awarded Fast Track and Orphan Drug status by the FDA and a registration study is underway. During 2020, we plan to continue to further develop duvelisib through our registration-directed Phase 2 PTCL study and other sponsored trials. We also expect that interim data for several ongoing investigator sponsored studies (ISTs) will be reported.

Defactinib, is a targeted inhibitor of Focal Adhesion Kinase (FAK). 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. CH5126766, is a first-in-class small molecule RAF/MEK inhibitor. Defactinib in combination with CH5126766 is being studied in an ongoing Phase 1 IST in patients with KRAS mutant advanced solid tumors, including ovarian cancer, non-small cell lung cancer (NSCLC) and colorectal cancer. Similar to COPIKTRA, both defactinib and CH5126766 are delivered orally and designed to be a potential therapy for patients to take at home under the advice of their physician. During 2020, we expect the results from the defactinib/CH5126766 IST to be reported.

In addition, defactinib is currently being investigated in combination with immunotherapeutic and other agents through ISTs. In 2020, it is planned to report results from certain ongoing dose escalation combination studies involving defactinib.

OUR FOCUS

We are focused on the development and commercialization of small molecules kinase inhibitors 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 2019, approximately 1.8 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, cell therapy, 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 tumor growth by disrupting the cell cycle leading to cell death. Chemotherapies are effective at killing cancer cells because cancer cells generally grow more rapidly than normal cells. However, chemotherapies also target fast-growing normal cells of the body, such as blood cells, hair follicles, and the cells lining the mouth, stomach, and intestines. As a result, they have a range of side effects and although the treatments may succeed at initially decreasing tumor burden, they ultimately fail to kill all the cancer cells and/or to effectively disrupt the tumor microenvironment (TME), 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. For example, the U.S. annual incidence, based on 2019 estimates from the National Cancer Institute's Surveillance, Epidemiology, and End Results Program (NCI; SEER), is that during the year there were approximately 74,200 new cases of indolent non-Hodgkin lymphoma (iNHL), 228,150 new cases of lung cancer and 56,770 new 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, 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 TME to enhance the efficacy of cancer treatment. Agents that can modulate the TME 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 is currently used to treat cancer patients with relapsed and refractory CLL/SLL and FL. COPIKTRA, and our product candidates, defactinib and CH5126766, are being further evaluated for the treatment of certain types of hematologic and solid cancers, CLL/SLL, iNHL, T-cell lymphoma, lung cancer, head and neck cancer, ovarian cancer, colorectal cancer, pancreatic cancer, mesothelioma, and other advanced cancers.

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

Hematologic malignancies are cancers in the cells of blood forming tissue (bone marrow) or in the cells of the immune system. CLL/SLL, a form of non-Hodgkin lymphoma (NHL) occurs in a type of white blood cells called lymphocytes, specifically B-cells. When most of the cancer cells are located in the bloodstream and the bone marrow, the disease is referred to as CLL. When the cancer cells are located mostly in the lymph nodes, the disease is called SLL. 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 4.9 per 100,000 men and women per year based on 2012-2016 cases and that the five-year relative survival rate from 2009 to 2015 for patients with CLL/SLL was approximately 85%.

Treatment of CLL/SLL has evolved over time from the initial use of monotherapy with alkylating agents (chlorambucil, bendamustine) and purine analogs (fludarabine) to immunotherapy (anti-CD52 monoclonal antibody alemtuzumab and anti-CD20 monoclonal antibody ofatumumab) and chemoimmunotherapy combinations (rituximab, ofatumumab and obinutuzumab in combination with purine and alkylating agents). Since 2014, novel targeted agents have been approved in the relapsed setting and include the Bruton's tyrosine kinase (BTK) inhibitor ibrutinib, the PI3K inhibitor idelalisib, in combination with rituximab, and the BCL-2 inhibitor venetoclax. While available therapies have been shown to be effective, the majority of CLL/SLL will eventually relapse or become refractory to treatment. In the relapsed or refractory population, other available therapies may not be appropriate for

some patients due to the patient's age, comorbidities, response to prior therapies, and underlying disease. In addition, some other available therapies have limitations due to route of administration (e.g., intravenous infusion) and the requirement for close monitoring within a hospital setting, which may prohibit their use in patients who have limited access to hospitals or clinics. In consideration of eventual relapse, and the potential for treatment-associated toxicities or treatment-resistance, as well as patient convenience and accessibility, additional options are needed to treat patients with relapsed or refractory CLL/SLL.

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. Currently, COPIKTRA is also being evaluated in combination with chemotherapy, or venetoclax for the treatment of patients with CLL/SLL.

Follicular Lymphoma

FL is typically a slow growing or indolent form of NHL that arises from B-lymphocytes. FL accounts for approximately 20% of case of NHL. Common symptoms of FL include enlargement of the lymph nodes in the neck, underarms, abdomen, or groin, as well as fatigue. Typically, patients with FL have no obvious symptoms of the disease at diagnosis and present only with an enlarged lymph node on exam or found incidentally on the imaging study. The NCI estimates that the number of new incidences of FL was 2.7 per 100,000 men and women per year based on 2012-2016 cases and that the five-year relative survival rate from 2009 to 2015 for patients with FL was approximately 88%. Despite recent improvements in survival, FL remains an incurable disease.

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. The standard first line therapies for FL are rituximab, cyclophosphamide, doxorubicin, vincristine and prednisone (R-CHOP); rituximab, cyclophosphamide, vincristine, and prednisone (R-CVP) and rituximab and bendamustine. In most patients, these rituximab-containing regimens achieve remissions lasting 4-7 years. Rituximab is used alone as a first-line treatment for patients whose frailty or comorbidities prelude cytotoxic therapy. Single-agent rituximab also may be beneficial as maintenance therapy for patients who experience partial remission with first-line regimen. Second-line treatments for follicular lymphoma include re-exposure to common first-line regimens, the radiopharmaceutical yttrium Y 90–ibritumomab tiuxetan, and the recently approved combination of bendamustine and obinutuzumab, a second-generation anti-CD20 antibody. Patients with FL often undergo multiple courses of chemoimmunotherapy, increasing the risk of serious cardiac toxicity, immunosuppression, and myelosuppression, which is particularly problematic in elderly patients. The choice of third-line treatment for relapsed/refractory follicular lymphoma depends largely on a patient's age, how long the most recent remission lasted, and how aggressive the disease is.

There have been only incremental advances in treatment options for FL beyond chemotherapy or immunotherapies like the antibodies against CD20, such as rituximab and obinutuzumab, and the overall clinical outlook for patients still remains poor. The 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. Currently, COPIKTRA is also being evaluated in combination with Nivolumab, and in an intermittent dosing regimen for the treatment of patients with FL.

Peripheral T-Cell Lymphoma

Peripheral T-cell lymphomas constitute approximately 4% of all NHLs diagnosed in the United States. The incidence of peripheral T-cell lymphoma increases with age, with the median age at diagnosis at 60 years. The disease course is very aggressive and is associated with poor prognosis. Current treatment strategies are largely unsatisfactory in both first-line and refractory/relapsed. Without treatment, PTCL survival is measured in months. Most patients will relapse with first-line treatment and will not achieve remission of their disease. The approach to

treat PTCL has traditionally been similar to that for diffuse large B-cell lymphoma. However, outcomes are poor when PTCL is treated according to strategies established for aggressive B-cell lymphomas, with early relapse, progression free survival of less than 1 year, and overall survival of less than 2 years. Even with current standard anthracycline-based chemotherapy, the 5 year survival rate is low, which presents the need for new therapies.

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 four 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), and cutaneous T-cell lymphoma (CTCL) – account for approximately 88% 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, and prednisone), CHOEP (cyclophosphamide, doxorubicin, vincristine, etoposide, and 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.

Historically, patients with relapsed disease have had a dismal prognosis, with a median survival of less than 6 months. Patients with relapsed disease are usually treated with combination chemotherapy such as ICE (ifosfamide, carboplatin and etoposide) or other combination regimens, followed by stem cell transplantation. However, some regimens might not be suited for everyone because of their high toxicity levels.

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. Currently, COPIKTRA is being evaluated as monotherapy or in combination with romidepsin or bortezomib for the treatment of patients with PTCL.

Head and Neck Cancer

Cancers that are known collectively as head and neck cancers usually begin in the squamous cells that line the moist, mucosal surfaces inside the head and neck (for example, inside the mouth, the nose, and the throat). These squamous cell cancers are often referred to as head and neck squamous cell carcinoma (HNSCC). Head and neck cancers can also begin in the salivary glands, but salivary gland cancers are relatively uncommon. Salivary glands contain many different types of cells that can become cancerous, so there are many different types of salivary gland cancer. In the U.S, there are approximately 50,000 new cases of HNSCC and more than 10,000 deaths per year.

HNSCC represents over 90% of head and neck malignancies. Although the majority of patients present with locoregionally advanced disease, the clinical course is characterized by a high recurrence rate approaching 50%, as well as the development of distal metastases. The current standard of care for locally recurrent disease (without surgical or radiation treatment options) and/or metastatic disease in the first-line setting has been platinum-based doublet chemotherapy with cetuximab. In 2016, the FDA approved two immunotherapeutic agents, nivolumab and pembrolizumab, for the treatment of patients with R/M HNSCC refractory to platinum-based therapy. In 2019, the FDA approved pembrolizumab for the first-line treatment of patients with unresectable recurrent/metastatic (R/M) HNSCC. For frontline therapy, pembrolizumab was approved for use in combination with platinum and fluorouracil for all patients with R/M HNSCC and as a single agent for patients whose tumors express PD-L1 with a combined positive score (CPS) ≥1 as determined by an FDA-approved test.

Despite the recent approval of pembrolizumab and nivolumab, only a small subset of patients appears to derive benefit from these anti-PD-1 therapies. Although the efficacy may vary by PD-L1 expression or human papillomavirus (HPV) status as well as the number of prior therapies, typically the ORR is approximately 15% in the second-line or greater setting and somewhat higher in the first-line setting. The majority of responses in either setting are partial responses (PRs). Thus, there remains a large unmet medical need for better therapy options in patients with R/M HNSCC. Currently, duvelisib is being evaluated in patients who are eligible for pembrolizumab monotherapy based on the current pembrolizumab prescribing information.

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 NCI estimates that the number of new incidences of ovarian cancer was 11.4 per 100,000 women per year based on 2012-2016 cases and that the five-year relative survival rate from 2009 to 2015 for patients with ovarian cancer was approximately 48%.

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. Currently, defactinib is being evaluated in combination with CH5126766 for the treatment of low grade serous ovarian cancer (LGSOC), and in combination with platinum and taxane for the treatment of patients with high grade serous ovarian cancer (HGSOC).

Colorectal Cancer

Colorectal cancer (CRC), also known as bowel cancer, colon cancer, or rectal cancer, is the development of cancer from the colon or rectum (parts of the large intestine). One in twenty people will be diagnosed with colorectal cancer and colorectal cancer is the second leading cause of cancer death among men and women combined in the United States. The NCI estimates that the number of new incidences of CRC was 38.6 per 100,000 men and women per year based on 2012-2016 cases and that the five-year relative survival rate from 2009 to 2015 for patients with CRC was approximately 64%. The individual likelihood of survival depends on how advanced the cancer is, whether or not all the cancer can be removed with surgery and the person's overall health.

Treatments used for colorectal cancer may include some combination of surgery, radiation therapy, chemotherapy and targeted therapy. Cancers that are confined within the wall of the colon may be curable with surgery, while cancer that has spread widely is usually not curable, with management being directed towards improving quality of life and symptoms. Currently, defactinib is being evaluated in combination with CH5126766 for the treatment of patients with RAS mutant colorectal cancer.

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 NCI estimates that the number of new incidences of lung cancer was 54.9 per 100,000 men and women per year based on 2012-2016 cases and that the five-year relative survival rate from 2009 to 2015 for patients with lung cancer 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. Currently, defactinib is being evaluated in combination with pembrolizumab or in combination with CH5126766 for the treatment of patients with NSCLC cancer.

Pancreatic Cancer

In 2019, 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. The NCI estimates that the number of new incidences of pancreatic cancer was 12.9 per 100,000 men and women per year based on 2012-2016 cases. 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 2009 to 2015. The median age for diagnosis is 70 with the disease affecting males slightly more than females.

The prognosis for pancreatic cancer is extremely poor as shown by the survival rate, which indicates the need for new treatments. 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 TME. Currently, defactinib is being evaluated in combination with pembrolizumab for the treatment of patients with pancreatic cancer.

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. The average life expectancy for mesothelioma patients is approximately 12 to 22 months. 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. There are four types of mesothelioma, with pleural mesothelioma accounting for approximately 75% of all mesothelioma cases.

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. Although significant progress has been made in the treatment of many malignancies, mesothelioma remains a therapeutic challenge, with disappointing results even when the most aggressive therapies are used. Given the lack of effective treatments, a substantial unmet need exists for improved first-line regimens and efficacious second-line treatments. Currently, defactinib is being evaluated in combination with pembrolizumab for the treatment of patients with mesothelioma.

OUR STRATEGY

COPIKTRA, defactinib, and CH5126766 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.
- Continuing to develop and explore combination of defactinib and CH5126766 and further define the initial registration-directed study for defactinib and CH5126766 in combination.
- Expanding the indications in which COPIKTRA, defactinib and CH5126766 may be used. In parallel with the CLL/SLL, iNHL, PTCL, NSCLC, HNSCC, ovarian cancer, pancreatic cancer, and mesothelioma trials and studies that are currently being conducted, we plan to pursue additional disease indications to expand the potential of our product and product candidates.
- Supporting our third party collaborators outside of the United States to successfully continue on their pathway to development milestones and regulatory approval for duvelisib in their respective territories.
- Advancing our product candidates through clinical development. In addition to ongoing clinical trials evaluating duvelisib and CH5126766 as single agents, clinical studies are currently ongoing to evaluate duvelisib, defactinib and CH5126766 in combinations with other agents in several hematologic and solid tumor indications.
- 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 continue to 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 candidates 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, defactinib and CH5126766, which continue 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 CLL/SLL after at least two prior therapies and relapsed or refractory 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 ≥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 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, marginal zone lymphoma (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

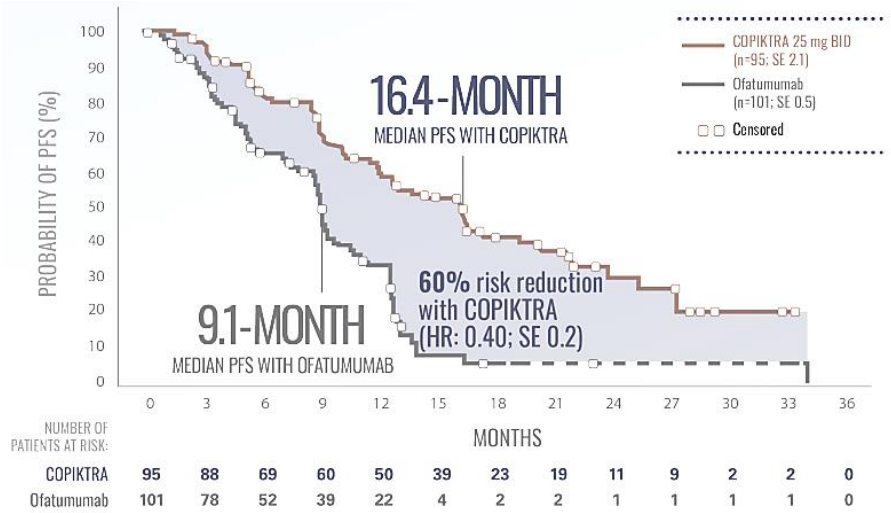
The CLL/SLL indication for COPIKTRA is based on data from a subset of patients in the DUO trial who had received two or more prior lines of therapy. These 196 patients were the majority of patients enrolled in DUO. The sub-analysis data included in the COPIKTRA label resulted in a median PFS by central 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)

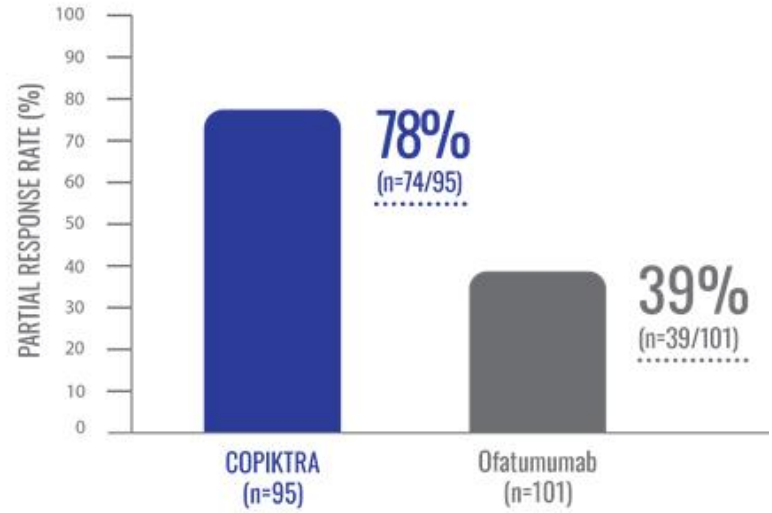
Outcome per IRC	COPIKTRA N = 95	Ofatumumab N=101
PFS		
Number of events, n (%)	55 (58%)	70 (69%)
Progressive disease	44	62
Death	11	8
Median PFS (SE), months [a]	16.4 (2.1)	9.1 (0.5)
Hazard Ratio (SE), [b] COPIKTRA/ofatumumab	0.40 (0.2)	
Response Rate		
ORR n (%) [c]	74 (78%)	39 (39%)
CR	0 (0%)	0 (0%)
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(hazard ratio) = 0.2*
[c] *IWCLL or revised IWG response criteria, with modification for treatment-related lymphocytosis*

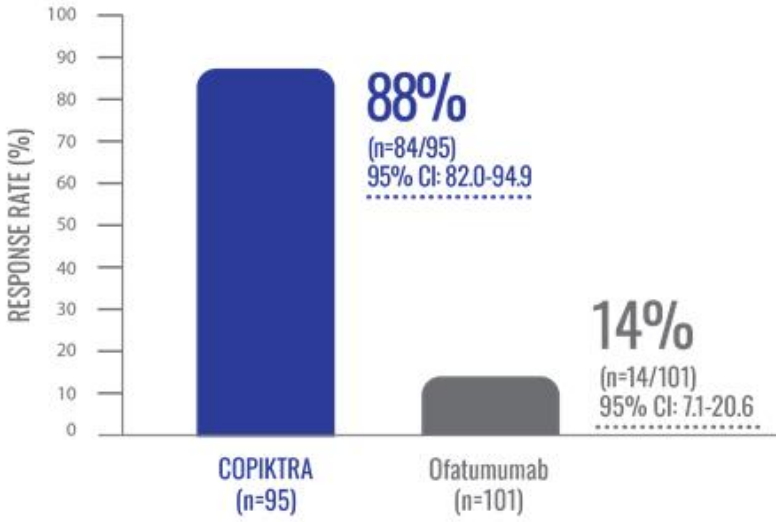
Kaplan-Meier Curve of PFS per IRC In Patients with at Least 2 Prior Therapies (DUO)



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 FL after at least two prior systemic therapies.

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 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 include all patients from the DUO and DYNAMO studies.

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

Adverse Reactions	COPIKTRA 25 mg BID (N = 442)	
	Grade ≥ 3 ***n (%)***	***Any Grade*** ***n (%)***
Neutropenia †	132 (30%)	151 (34%)
Diarrhea or colitis †[a]	101 (23%)	222 (50%)
Pneumonia †[b]	67 (15%)	91 (21%)
Anemia †	48 (11%)	90 (20%)
Rash †[c]	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
[a] *Diarrhea or colitis includes the preferred terms: colitis, enterocolitis, colitis microscopic, colitis ulcerative, diarrhea, diarrhea hemorrhagic*
[b] *Pneumonia includes the preferred terms: All preferred terms containing "pneumonia" except for "pneumonia aspiration"; bronchopneumonia, bronchopulmonary aspergillosis*
[c] *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%).

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.

Phase 1/2 Study Investigating Duvelisib and Venetoclax in Patients with Relapsed or Refractory CLL/SLL

The Phase 1/2 Investigator-sponsored study, with Matthew S. Davids, MD, Associate Director, Center for Chronic Lymphocytic Leukemia at Dana-Farber Cancer Institute, as Principal Investigator for the trial, is exploring duvelisib in combination with venetoclax in relapsed or refractory CLL/SLL. In the study, 12 patients were enrolled and received oral duvelisib and oral venetoclax. The primary endpoints of the study are dose limiting toxicities, maximum tolerated dose and identification of the recommended Phase 2 dose. Secondary endpoints include pharmacokinetics and preliminary efficacy. Duvelisib plus venetoclax demonstrated promising clinical activity, and a manageable tolerability profile, and a recommended Phase 2 dose of 400 mg of venetoclax was determined for this regimen.

Among the 12 evaluable patients, 11 achieved a response for an ORR of 92%, including four (33%) complete responses and seven (58%) partial responses. Four patients had undetectable minimum residual disease in the peripheral blood and bone marrow, including two patients with a complete response. The most common Grade 1 and 2 adverse events were fatigue (92%), hyperglycemia (83%), anemia (67%), and thrombocytopenia (67%). The most common Grade ≥3 adverse events were neutropenia (84%), hypocalcemia (50%), and hypophosphatemia (25%). No dose limiting toxicities were observed, and the recommended phase 2 dose of venetoclax was identified as 400mg once daily when given with standard dose duvelisib 25mg twice daily. This study is now in a Phase 2 portion in CLL/SLL and Richter's Syndrome and is currently accruing new patients.

DUVELISIB TREATMENT IN T-CELL LYMPHOMA

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 and five partial responses. Responses were seen across the spectrum of PTCL subtypes, including compete responses and partial responses 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 partial responses. 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 the first quarter of 2018, an open-label, multicenter, Phase 2 clinical trial (PRIMO) evaluating the efficacy and safety of duvelisib in patients with relapsed or refractory PTCL was initiated.

PRIMO STUDY

The PRIMO study is a multi-center, open-label, registration-directed Phase 2 study evaluating duvelisib in patients with relapsed or refractory PTCL that is expected to enroll approximately 120 patients. In the dose optimization portion of the study, patients were randomized to receive duvelisib 25mg twice daily with an option for dose escalation (cohort 1) or duvelisib 75mg twice daily continuously (cohort 2) until disease progression or unacceptable toxicity. The primary endpoint of the study was investigator-assessed overall response rate (ORR), and secondary endpoints included DOR and safety.

Preliminary results from the Dose Optimization portion the PRIMO study were presented at the 2019 Annual Meeting of the American Society for Hematology conference (ASH 2019). A total of 33 patients (cohort 1, n=20; cohort 2, n=13) were treated in the dose optimization/dose selection phase. Investigator-assessed ORRs were 35% in cohort 1 and 54% in cohort 2, with a complete response rate of 25% and 31% in cohort 1 and cohort 2, respectively. ORR, as assessed by blinded independent central review was 40% in cohort 1 and 62% in cohort 2. Thirteen of 20 patients in cohort 1 and all patients in cohort 2 were able to complete one cycle of therapy. Seven patients in cohort 1 discontinued therapy early due to disease progression and/or toxicity. Most responses were

observed at the end of cycle 1 (cycle=28 days). At a median follow-up of 21.4 weeks, the majority of responders were still in response at the time of their last assessment. The most common (≥ 4 patients) Grade ≥3 adverse events in all patients receiving duvelisib were neutropenia, thrombocytopenia, diarrhea, rash/maculopapular, lymphocytopenia, pneumonia and sepsis. Serious adverse events occurring in ≥ 2 patients were colitis, diarrhea, abdominal pain, pyrexia, sepsis, pneumonia, hyponatremia, rash/maculopapular, dyspnea, and respiratory failure.

Based on these efficacy and safety data, the investigators have elected to investigate duvelisib starting at 75mg twice daily for two cycles, followed by 25mg twice daily, during the dose expansion portion of the study which is currently ongoing. The expansion portion of the study is being conducted in the United States, Europe, and Japan.

During 2017, the FDA granted Fast Track designation for the treatment of patients with PTCL, who have received at least one prior therapy. In addition, the FDA granted duvelisib orphan drug designation for the treatment of T-cell lymphoma.

DEFACTINIB and CH5126766

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

CH5126766 is an orally available unique small molecule RAF/MEK inhibitor. Standard MEK inhibitors (e.g. trametinib) paradoxically induce MEK phosphorylation (pMEK) by relieving ERK-dependent feedback inhibition of RAF which may limit their efficacy. By inhibiting RAF phosphorylation of MEK, CH5126766 has the advantage of not inducing pMEK. This unique mechanism of CH5126766 enables more effective inhibition of ERK signaling and may confer enhanced therapeutic activity against ERK-dependent, RAS or BRAF mutant tumors. CH5126766 has been studied in over 150 patients and has shown a manageable safety profile to date. Initial signs of activity have been observed in clinical studies as a monotherapy in KRAS mutant, NSCLC, endometrial and ovarian cancers, in BRAF mutant ovarian cancer, and in RAS mutant multiple myeloma.

Defactinib and CH5126766 have each shown independent clinical activity against RAS mutant cancers.

Phase I study (FRAME) of defactinib in combination with a dual RAF/MEK inhibitor (CH5126766) in patients with advanced solid tumors.

Defactinib has shown a high level of synergy in combination with CH5126766 in preclinical models.

A dose escalation/dose expansion study is underway to evaluate the combination of defactinib and CH5126766 in patients with advanced solid tumors, including ovarian cancer, NSCLC, and colorectal cancer. The study was initiated in 2017 and has a planned enrollment of approximately 80 patients. This is a multi-center study sponsored by The Institute of Cancer Research (UK) under the direction of the chief investigator Professor Udai Banerji. There are two parts to this study: the dose escalation phase and the expansion phase. During the dose escalation phase, the recommended Phase 2 dose has been determined and expansion cohorts are underway. Defactinib is generally well tolerated, and has a non-overlapping safety profile relative to CH5126766 and a manageable all-oral combination regimen has been defined. Initial clinical data with the combination are promising for both objective response rate and durability. We intend to explore the breadth of this combination against KRAS mutant cancers and we expect the clinical results will be reported in 2020.

FAK inhibition effects on the tumor microenvironment

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 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 inhibitor with checkpoint inhibitors in the clinic for pancreatic and other solid tumors.

Phase 1/2 study with Cancer Research United Kingdom (CRUK) and Merck & Co. to evaluate combination therapy with pembrolizumab.

In September 2016, we announced a 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.

Patents

Our patent portfolio includes issued and pending applications worldwide. These patent applications fall into four categories: (1) PI3K inhibition program; (2) FAK inhibition program; (3) RAF/MEK inhibition program and (4) 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 patent 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, if 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 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.

RAF/MEK inhibition program

We have exclusively licensed a portfolio of three patent families owned by Chugai Pharmaceutical Co., Ltd. (Chugai). The first patent family has claims directed to the composition of matter of CH5126766, and includes granted patents in the United States, Australia, Canada, China, Europe and Japan, that are expected to expire in February 2027. The second patent family has one pending international application with claims directed to a dosing protocol of CH5126766. Any patent application claiming priority to or the benefit of this international patent application, if issued, would be expected to expire in May 2038. The third patent family has claims directed to method of making CH5126766 and includes granted patents in Europe, Japan, and the United States that are expected to expire in September 2032.

Patent Term

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

The term of a United States patent may be eligible for patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Act, to account for at least some of the time the drug is under development and regulatory review after the patent is granted. With regard to a 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 CLL/SLL after at least two prior therapies and relapsed or refractory 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.

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

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

On March 5, 2019, Infinity and Healthcare Royalty Partners III, L.P. (HCR) entered into a purchase and sale agreement, in which HCR paid Infinity a $30.0 million upfront payment and is entitled to receive up to $20.0 million in potential milestone payments from Infinity. In exchange HCR has received the right to receive the royalties due to Infinity from us under the license agreement. As a result, we now pay royalties previously due to Infinity to HCR. We will continue to pay Infinity for the royalties due to MICL and Purdue described above.

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.

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.

Sanofi

On July 25, 2019, we entered into a license and collaboration agreement with Sanofi (the Sanofi Agreement), under which we granted exclusive rights to Sanofi to develop and commercialize products containing duvelisib in Russia, the Commonwealth of Independent States (CIS), Turkey, the Middle East and Africa (collectively the "Sanofi Territory") for the treatment, prevention, palliation or diagnosis of any oncology indication in humans or animals.

Under the terms of the Sanofi Agreement, Sanofi received the exclusive right to develop and commercialize products containing duvelisib in the Sanofi Territory under mutually agreed upon development and commercialization plans at Sanofi's own cost and expense. In addition, Sanofi received certain limited manufacturing rights in the event the Company is unable to manufacture or supply sufficient quantities of duvelisib or products containing duvelisib to Sanofi during the term of the Sanofi Agreement. We retained all rights to duvelisib outside the Sanofi Territory, except for those territories previously and exclusively licensed to other parties.

Sanofi paid us an upfront, non-refundable payment of $5.0 million in August 2019. We are also entitled to receive aggregate payments of up to $42.0 million if certain regulatory and commercial milestones are successfully achieved. Sanofi is obligated to pay us double-digit royalties on net sales of products containing duvelisib in the Sanofi Territory, subject to reduction in certain circumstances. Sanofi and we have made customary representations and warranties and have agreed to certain customary covenants, including confidentiality and indemnification.

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

Pfizer Inc.

On July 11, 2012, we entered into a license agreement (the Pfizer 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 Pfizer 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 Pfizer 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 Pfizer 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 Pfizer Agreement in connection with an insolvency event involving the other party or a material breach of the Pfizer Agreement by the other party that remains uncured for a specified period of time. If the Pfizer 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.

Chugai Pharmaceutical Co., Ltd. (Chugai)

On January 7, 2020, we entered into a license agreement with Chugai (the Chugai Agreement)) whereby Chugai granted us an exclusive worldwide license for the development, commercialization and manufacture of products containing CH5126766, a dual RAF/MEK inhibitor.

Under the terms of the Chugai Agreement, we received an exclusive right to develop and commercialize products containing CH5126766 at our own cost and expense. We are required to pay Chugai a non-refundable payment of $3.0 million which was paid in February 2020. We are further obligated to pay Chugai double-digit royalties on net sales of products containing CH5126766, subject to reduction in certain circumstances. Chugai also obtained opt back rights to develop and commercialize CH5126766 (a) in the European Union, which option may be exercised through the date we submit a NDA to the FDA for a product which contains CH5126766 as the sole active pharmaceutical ingredient and (b) in Japan and Taiwan, which option may be exercised through the date we receive marketing authorization from the FDA for a product which contains CH5126766 as the sole active pharmaceutical ingredient. As consideration for executing either option, Chugai would have to make a payment to us calculated on the Company's development costs to date. Chugai and we have made customary representations and warranties and have agreed to certain customary covenants, including confidentiality and indemnification.

Unless earlier terminated, the Chugai Agreement will expire upon the fulfillment of our royalty obligations to Chugai for the sale of any products containing the CH5126766, which royalty obligations expire on a product-by-product and country-by-country basis, upon the last to occur, in each specific country, of (a) expiration of valid patent claims covering such product or (b) 12 years from the first commercial sale of such product in such country.

We may terminate the Chugai Agreement upon 180 days' written notice. Subject to certain limitations, Chugai may terminate the Chugai Agreement upon written notice if we challenge any patent licensed by Chugai to us under the Chugai Agreement. Either party may terminate the license agreement in its entirety with 120 days' written notice for the other party's material breach if such party fails to cure the breach. Either party may also terminate the Chugai Agreement in its entirety upon certain insolvency events involving the other party.

COMPETITION

The biotechnology and pharmaceutical industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. While we believe that our technology, development experience and scientific knowledge provide us with competitive advantages, we face potential

competition from many different sources, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions and governmental agencies and public and private research institutions. Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future.

Many of our competitors may have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical, biotechnology and diagnostic industries may result in even more resources being concentrated among a smaller number of our competitors. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

The key competitive factors affecting the success of all our product candidates, if approved, are likely to be their efficacy, safety, side effects, 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. To the extent our product candidates are ultimately used in combination with or as an adjunct to existing drug or other 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;
- Bayer AG, which has received approval from the FDA of copanlisib for the treatment of patients with relapsed FL and which we believe has ongoing Phase 1/2 and Phase 3 trials of copanlisib;
- 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 umbralisib;
- Incyte Corporation, which we believe is conducting a Phase 1 and 2 clinical trials of parsaclisib;
- MEI Pharma, which we believe is conducting Phase 1b and Phase 2 clinical trials of ME-401;
- Rhizen Pharmaceuticals, which we believe is conducting Phase 1 and Phase 2 clinical trials for tenalisib;
- Hutchison MediPharma, which we believe is conducting Phase 1 and Phase 2 clinical trials for HMPL-689; and
- Shanghai Yingli Pharmaceutical Co., which we believe is conducting Phase 1 and Phase 2 clinical trials for linperlisib.

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), B-lymphocyte antigen CD-19, programmed death 1/ligand 1 (PD-1/PD-L1), Enhancer of Zeste Homolog 2 (EZH2), Cluster of Differentiation 79B antibody-drug conjugate (CD79B ADC), anti-CD-47, CD20xCD3 bi-specific antibodies, Janus Kinase (JAK), 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, and 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 (a BMS company), which has received FDA approval of lenalidomide, an immunomodulator, for the treatment of patients with multiple myeloma, MCL, and myelodyplastic 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;
- Beigene, which we believe is conducting Phase 3 trials of zanubrutinib, a BTK inhibitor, in patients with CLL;

- Epizyme, which has submitted a NDA for tazemetostat (EZH2i) for the treatment of patients with FL; and

- Bristol Myers Squibb, which we believe is conducting Phase 1 and Phase 2 trials of liso-cel (CD-19 CAR T).

FAK and RAF/MEK inhibition programs

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 Corporation, Sanofi-Aventis U.S. LLC, GlaxoSmithKline plc, Boehringer Ingelheim GmbH, Pfizer Inc. and others.

RAF/MEK inhibition program

There are other companies with approved RAF and/or MEK inhibitors with FDA approval in the market. Such companies include:

- Novartis AG, which has received FDA approval for dabrafenib, a RAF inhibitor, in combination with trametinib, a MEK inhibitor, for treatment of patients with unresectable or metastatic melanoma with BRAF V600E or V600K mutations, adjuvant treatment for melanoma with BRAF V600E or V600K mutations and involvement of lymph nodes following complete resection, metastatic NSCLC with BRAF V600E or V600K mutations and locally advanced or metastatic anaplastic thyroid cancer with BRAF V600E mutation;

- Pfizer, through its acquisition of Array BioPharma, Inc, has received FDA approval for encorafenib, a RAF inhibitor, in combination with binimetinib, a MEK inhibitor, for treatment of patients with unresectable or metastatic melanoma with a BRAF V600E or V600K mutation; and

- Genentech, Inc. a member of the Roche Company, which has received FDA approval for vemurafenib, a RAF inhibitor, in combination with cobimetinib, a MEK inhibitor, to treat patients with unresectable or metastatic melanoma with a BRAF V600E or V600K mutation.

FAK and RAF/MEK Combination

There are other companies working to develop therapies against KRAS-mutant cancers. We believe the following companies have ongoing phase 2 trials for treatment of patients with KRAS-mutant cancers: Amgen, Inc. (AMG-510) Mirati Therapeutics, Inc. (MTRX-849), Eli Lily and Company (LY-3499446), and Silenseed LTD (siG12D LODER).

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 two contract manufacturing organizations (CMO) for the production of duvelisib raw materials, one CMO for the production of duvelisib drug substance, one CMO for the production of duvelisib 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 production of duvelisib drug substance and drug product. 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 substance, drug product and packaging services 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 relapsed and refractory 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 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 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 $2.9 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 application should be approved. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

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

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

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

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

Fast track designation

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

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

Priority review

Under FDA policies, a product candidate may be eligible for priority review, or review within a six-month time frame from the time a complete application is accepted for filing. Products regulated by the FDA's Center for Drug Evaluation and Research (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

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.

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;

- 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 require manufacturers to adopt compliance programs that incorporate certain standards 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, 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. Additionally, coverage and reimbursement for drug products can differ significantly from payor to payor. One third-party payor's decision to cover a particular drug product or service does not ensure that other payors will also provide coverage for the drug product, or will provide coverage at an adequate reimbursement rate.

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, under the Medicare Coverage Gap Discount Program, 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. 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 existing controls and measures, could limit payments for pharmaceuticals such as COPIKTRA and 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.

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 eliminated the tax penalty for individuals who do not maintain mandated health insurance coverage beginning in 2019.

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 resolution of the litigation, which could take some time, the Healthcare Reform Act is still operational in all respects. In December 2019, a federal appeals court agreed that the individual mandate provision was unconstitutional but remanded the case back to the district court to assess more carefully whether any provisions of the Healthcare Reform Act were severable and could survive. In March, the Supreme Court agreed to hear the case. 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. As another example, legislation passed in 2019 revised how certain prices reported by manufacturers under the Medicaid Drug Rebate Program are calculated, a revision that is reported to potentially increase rebates paid by manufacturers by approximately $3 billion over the next ten years.

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, 2019, we had 135 full-time equivalent employees, including a total of 13 employees with M.D. or Ph.D. degrees, and 6 part-time employees. Of the full-time employees, 36 employees are engaged in research and development activities. None of our employees are represented by a labor union or covered by a collective bargaining agreement. We consider our relationship with our employees to be good.

BUSINESS—EXECUTIVE OFFICERS OF THE REGISTRANT

The following table sets forth the name, age and position of each of our executive officers as of February 29, 2020.

Name	Age	Position
Brian Stuglik	60	Chief Executive Officer
Daniel Paterson	58	President, Chief Operating Officer
Robert Gagnon	45	Chief Business and Financial Officer

Brian Stuglik, age 60, has served as our Chief Executive Officer since July 2019 and as a member of our Board of Directors since September 2017. Mr. Stuglik founded Proventus Health Solutions in January 2016 and has over three decades of experience in U.S. and international pharmaceutical development, product strategy, and commercialization. Prior to founding Proventus Health Solutions, Mr. Stuglik served as the Vice President and Chief Marketing Officer for the oncology division of Eli Lilly and Company, from 2009 to December 2015. Mr. Stuglik received a Bachelor of Science in Pharmacy from Purdue University and holds memberships in the American Society of Clinical Oncology, the American Association of Cancer Research, and the International Association for the Study of Lung Cancer.

Daniel Paterson, age 58, has served as our President since June 2019 in addition to serving 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. Mr. Paterson holds a B.A. in Biology from Boston University, and attended the Northeastern University Graduate Pharmacology program.

Robert Gagnon, age 45, joined Verastem as Chief Financial Officer in 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. Mr. Gagnon holds an M.B.A from the MIT Sloan School of Management and a Bachelor of Arts degree in accounting from Bentley College.

OUR CORPORATE INFORMATION

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

ADDITIONAL INFORMATION

We maintain a website at www.verastem.com. We make available, free of charge on our website, our annual 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.

ITEM 1A. Risk Factors.

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

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), Pfizer, Inc. (Pfizer) and Chugai Pharmaceutical Co., Ltd (Chugai) to research, develop, commercialize, and manufacture products in oncology indications containing duvelisib, defactinib, and CH5126766, respectively. The success of this strategy depends partly upon our ability to identify, select, discover and acquire promising pharmaceutical product candidates and products. The process of proposing, negotiating and implementing a license or acquisition of a product candidate or approved product is lengthy and complex. Other companies, including some with substantially greater financial, marketing and sales resources, may compete with us for the license or acquisition of product candidates and approved products. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We have limited resources to identify and execute the acquisition or in-licensing of third-party products, businesses and technologies and integrate them into our current infrastructure. Moreover, we may devote resources to potential acquisitions or in-licensing opportunities that are never completed, or we may fail to realize the anticipated benefits of such efforts. We also may be unable to license or acquire the relevant compound or product candidate on terms that would allow us to make an appropriate return on our investment. Any product candidate that we acquire may require additional development efforts prior to commercial sale, including manufacturing, pre-clinical testing, extensive clinical testing and approval by the FDA and applicable foreign regulatory authorities. All product candidates are prone to risks of failure typical of pharmaceutical product development.

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

- exposure to unknown liabilities;
- disruption of our business and diversion of our management's time and attention to develop acquired products, product candidates or technologies;
- higher than expected acquisition and integration costs;
- increased amortization expenses; and
- incurrence of substantial debt, dilutive issuances of securities or depletion of cash to pay for acquisitions.

Future business acquisitions may also entail certain additional risks, such as:

- difficulty in combining the operations and personnel of any acquired businesses with our operations and personnel;
- impairment of relationships with key suppliers or customers of any acquired businesses due to changes in management and ownership; and
- inability to motivate key employees of any acquired businesses.

If 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;
- 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.

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.

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, FAK and RAF/MEK signaling pathways and disrupt the tumor microenvironment is an emerging field and, consequently, there is still uncertainty about whether COPIKTRA, defactinib and CH5126766 are effective in improving outcomes for patients with cancer.

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, FAK or RAF/MEK 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.

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

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

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

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

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 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, Celgene Corporation, Astellas Pharma Inc., GlaxoSmithKline plc, Boehringer Ingelheim GmbH 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.

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 are seeking regulatory approval for COPIKTRA and intend to seek regulatory approval for our product candidates in countries outside of the United States and expect that these countries will be important markets for our products, if approved. In November 2019, we submitted a marketing authorization application to the European Medicines Agency for COPIKTRA. 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.

Although we have invested a significant amount of time, resources and effort on the commercialization of COPIKTRA, we may not be successful in the commercialization 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 continue to invest 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;
- 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 marketed drug by a wide 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 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.

If serious adverse or unexpected side effects are identified during the commercialization of COPIKTRA or development of our product candidates, we may need to abandon or limit the commercialization of COPIKTRA and abandon or limit our development of some of our 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 have implemented 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 ≥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. CH5126766 is in a Phase 1 clinical trial. For both defactinib and CH5126766, the toxicities reported to date have been predictable and manageable.

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 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.

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.

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 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.

Business or economic disruptions or global health concerns could seriously harm sales of COPIKTRA, our development efforts and increase our costs and expenses.

Broad-based business or economic disruptions could adversely affect our ongoing or planned research and development activities and sales of COPIKTRA. For example, in December 2019 an outbreak of a novel strain of coronavirus originated in Wuhan, China, and has since spread to a number of other countries, including the United States. To date, this outbreak has already resulted in extended shutdowns of certain businesses in the Wuhan region and has had ripple effects to businesses around the world. Global health concerns, such as coronavirus, could also result in social, economic, and labor instability in the countries in which we or the third parties with whom we engage operate. We cannot presently predict the scope and severity of any potential business shutdowns or disruptions, but if we or any of the third parties with whom we engage, including the suppliers, clinical trial sites, regulators and other third parties with whom we conduct business, were to experience shutdowns or other business disruptions, our ability to conduct our business in the manner and on the timelines presently planned could be materially and negatively impacted. We further cannot presently predict the impact, if any, global health concerns, such as coronavirus, will have on sales of COPIKTRA. It is also possible that global health concerns such as this one could disproportionately impact the hospitals and clinical sites in which we conduct any of our clinical trials, which could have a material adverse effect on our business and our results of operation and financial condition.

Risks Related to Our License Agreements with Infinity, Pfizer and Chugai

If we do not realize the anticipated benefits of our license agreements with Infinity for the COPIKTRA program, Pfizer for the FAK program and Chugai for the dual RAF/MEK candidate program, our business could be adversely affected.

Our license agreements with Infinity for COPIKTRA, Pfizer for defactinib, and Chugai for CH5126766 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, defactinib and CH5126766 on our financial results relating to numerous matters, including:

- the cost of development and commercialization of COPIKTRA, defactinib and CH5126766; and
- other financial and strategic risks related to the license agreements with Infinity, Pfizer and Chugai.

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 agreements with Infinity, Pfizer and Chugai. 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 agreements with Infinity for COPIKTRA, Pfizer for defactinib, and Chugai for CH5126766 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, 2019, we had an accumulated deficit of $524.8 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, as amended, with Hercules Capital Inc. (Hercules), the issuance of our 5.00% Convertible Senior Notes due 2048 (the 2018 Notes) and the issuance of our 5.00% Convertible Senior Second Lien Notes due 2048 in exchange for a portion of the 2018 Notes (the 2019 Notes). As of December 31, 2019, there was $40.0 million available to borrow under the amended term loan facility with Hercules, subject to certain milestones and 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:

- continue our ongoing commercialization of COPIKTRA;
- continue our ongoing clinical trials with our product candidates, including with COPIKTRA, defactinib, and CH5126766';
- 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 and CH5126766.

We expect our existing cash resources at December 31, 2019, the proceeds from the sale of Common Stock in March 2020, and revenue we expect to generate from COPIKTRA will be sufficient to fund our current operating plan and capital expenditure requirements beyond the next twelve months from the issuance date of these financial statements. 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. 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.

Risks Related to Non-Cash Impairment

If our intangible asset becomes impaired, we may need to record significant non-cash impairment charges.

We have recorded a $22.0 million milestone payment, which became payable upon the FDA marketing approval on September 24, 2018 related to COPIKTRA pursuant to the amended and restated license agreement with Infinity as an intangible asset on our consolidated balance sheet. We assess our finite-lived intangible assets for impairment if indicators are present or changes in circumstance suggest that impairment may exist in accordance with *Accounting Standards Codification* 350 (ASC 350), *Intangibles – Goodwill and other.*

One potential indicator of impairment is whether there is a significant change in the manner in which we continue to commercialize COPIKTRA. On February 28, 2020, we announced a new strategic direction to accelerate the advancement of certain clinical development programs. We announced our primary focus will be on the development of CH5126766 in combination with defactinib, while we will also continue to advance the development of COPIKTRA for treatment of relapsed or refractory PTCL.

Based on our new strategic direction, we believe it is reasonably possible that we could record an impairment charge in the near term. Any such impairment charges, if significant, could adversely affect our financial position and results of operations.

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 (the Original Loan Agreement), which was subsequently amended in January 2018, March 2018, October 2018, April 2019 and November 2019 (the 2019 Term Loan Agreement). Under the 2019 Term Loan Agreement, Hercules will provide access to term loans with an aggregate principal amount of up to $75.0 million. As of December 31, 2019, there was $40.0M available to borrow in multiple tranches comprised of (i) a term loan in an amount of up to $15.0 million upon the Company generating cumulative net product revenues (as defined the 2019 Term Loan Agreement) of

either (a) $37.5 million on or before April 30, 2020 or (b) $50.0 million on or before June 30, 2020 and (ii) a term loan in an amount of up to $25.0 million available through December 31, 2021, subject to Hercules' approval and the satisfaction of certain other conditions. All obligations under the 2019 Term 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 2018 Notes. The 2018 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 2018 Indenture). The 2018 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 2018 Notes will mature on November 1, 2048, unless earlier repurchased, redeemed or converted in accordance with their terms.

In November and December 2019, we entered into privately negotiated agreements with a limited number of investors who are accredited investors (within the meaning of Rule 501 promulgated under the Securities Act of 1933, as amended (Securities Act)) and/or qualified institutional buyers (as defined in Rule 144A under the Securities Act to exchange approximately $121.7 million aggregate principal amount of the 2018 Notes for (i) approximately $66.9 million aggregate principal amount of the 2019 Notes and (b) an aggregate of approximately $12.1 million in cash. The 2019 Notes are governed by the terms of an indenture (the 2019 Indenture, and together with the 2018 Indenture, referred to as the Indentures). The 2019 Notes are senior secured obligations of the Company and bear interest at 5.00% per annum, payable semi-annually in arrears on May 1 and November 1 of each year. The 2019 Notes will mature on November 1, 2048, unless earlier repurchased, redeemed or converted in accordance with the terms. Collectively, the 2018 Notes and 2019 Notes are referred to as the Notes.

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 2019 Term Loan Agreement could result in an event of default that, if not cured or waived, would accelerate our obligation to repay this indebtedness. Such acceleration could also create an event of default under the Indentures whereupon both Hercules and the holders of the 2019 Notes could seek to enforce their respective security interests 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 2019 Term Loan Agreement, or the Indentures or breaching any covenants under the 2019 Term Loan Agreement, or the Indentures 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 Indentures prior to maturity in 2048. In the event of an acceleration of amounts due under the 2019 Term Loan Agreement, or the Indentures, 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 and the holders of the 2019 Notes could also exercise their rights to take possession and dispose of the collateral securing the term loans on a first priority basis and the 2019 Notes on a second priority basis, which collateral includes substantially all of our property other than our intellectual property. Our business, financial condition and results of operations could be

materially adversely affected as a result of any of these events. We are subject to certain restrictive covenants which, if breached, could have a material adverse effect on our business and prospects.

The 2019 Term Loan Agreement and the Indentures 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.

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.

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, failure or damage to a facility by natural disasters or public health crises, such as the recent coronavirus outbreak originating in Wuhan, China, 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 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, in Japan with Yakult Honsha Co., Ltd., and in Russia, the Commonwealth of Independent States, Turkey, the Middle East and Africa with Sanofi. We anticipate that we may seek to enter into a collaboration for marketing and commercialization of our product candidates in certain territories worldwide at the appropriate time in the future. Our likely collaborators for any collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. If we do enter into any such arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements.

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

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

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

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 sales of COPIKTRA, as well as the commercial launch and sales of 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. Furthermore, arrangements between manufacturers and specialty pharmacies and distributors as well as the provision of patient support services by or on behalf of manufacturers have been subject to government scrutiny under fraud and abuse laws. Like other manufacturers, we must ensure such arrangements are structured appropriately to mitigate the risk of challenge.

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, Pfizer, and Chugai, 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, Pfizer, and Chugai, 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 Chugai were to terminate its license agreement with us for any reason, we could lose our rights to CH5126766.

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, 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.

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 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 for use of COPIKTRA in T-Cell Lymphoma, 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.

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;
- 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;
- the federal anti-kickback prohibition known as Eliminating Kickbacks in Recovery Act or EKRA, enacted in 2018 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 and data privacy 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.

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. 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. In December 2019, a federal appeals court agreed that the individual mandate provision was unconstitutional but remanded the case back to the district court to assess more carefully whether any provisions of the Healthcare Reform Act were severable and could survive. In March, the Supreme Court agreed to hear the case. Pending resolution of the litigation, 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 2029. 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

We may experience difficulties in managing restructurings and restructuring activities may not be as effective as anticipated

On October 28, 2019, we committed to an operation plan to reduce overall operating expenses, including the elimination of approximately 40 positions. In addition on February 27, 2020, the Company committed to an operational plan to reduce overall operating expenses, including the elimination of approximately 31 positions.

The workforce reduction is designed to streamline operations, speed execution of the Company's clinical development of defactinib and CH5126766, and reflect a focused, account-based approach in the field. We may not realize, in full or in part, the anticipated benefits, savings and improvements in our cost structure from our restructuring efforts due to unforeseen difficulties, delays or unexpected costs. Furthermore, our restructuring plan may be disruptive to our operations. If we are unable to realize the expected operational efficiencies and cost savings from the restructuring, our operating results, which include net product revenue, and financial condition would be adversely affected. There can be no assurance that we will be successful in implementing our restructuring program. Future growth would impose significant added responsibilities on members of management, including the need to identify, recruit, maintain and integrate additional employees. Our future financial performance and our ability to commercialize COPIKTRA and develop our product candidates or additional assets will depend, in part, on our ability to effectively manage any future growth or restructuring, as the case may be.

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 Brian Stuglik, Chief Executive Officer, Daniel Paterson, our President and Chief Operating Officer, and Robert Gagnon, our Chief Business and Financial Officer. Although we have formal employment agreements with Brian Stuglik, Daniel Paterson, and Robert Gagnon, these agreements do not prevent them from terminating their employment with us at any time. We do not maintain "key person" insurance for any of our executives or other employees. The loss of the services of any of these persons could impede the achievement of our research, development and commercialization objectives.

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

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;
- 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 $0.83 through December 31, 2019. 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.

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

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

The delisting of our common stock would significantly affect the ability of investors to trade our common stock and negatively impact the liquidity and price of our common stock. In addition, the delisting of our common stock could materially adversely impact our ability to raise capital on acceptable terms or at all. Delisting from Nasdaq could also have other negative results, including the potential loss of confidence by our current or prospective third-party providers and collaboration partners, the loss of institutional investor interest, and fewer licensing and partnering opportunities.

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.

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 2018 Notes are effectively subordinated to our secured indebtedness and structurally subordinated to any liabilities of our subsidiaries.

The 2018 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 2018 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 2018 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 2018 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 2018 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 2018 Notes then outstanding. The 2018 Indenture governing the 2018 Notes does 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, including the Indentures, some of which may be secured debt. The 2019 Term Loan Agreement also restricts our ability and the ability of our subsidiaries to issue or incur additional indebtedness, including secured indebtedness, though if our loans under the 2019 Term Loan Agreement, mature or are repaid, we may not be subject to such restrictions under the terms of any subsequent indebtedness.

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, respectively 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 the 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 2019 Term Loan Agreement currently limits our ability to repurchase the Notes. Our failure to repurchase the Notes at a time when the repurchase is required by the Indentures governing the Notes would constitute a default under the Indentures. A default under the Indentures or the fundamental change itself could also lead to a default under the 2019 Term Loan Agreement, 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 2019 Term Loan Agreement 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 2019 Term Loan Agreement limits our ability to pay any cash amount upon repurchase of the Notes.

The 2019 Term Loan Agreement 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 Indentures governing the Notes and would permit holders of the Notes to accelerate our obligations under the Notes. A default under the Indentures or the fundamental change itself could also lead to a default under the 2019 Term Loan Agreement, 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.

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, 2020 there were 11 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 $2.79. The number of holders of record does not include beneficial owners whose shares are held by nominees in street name.

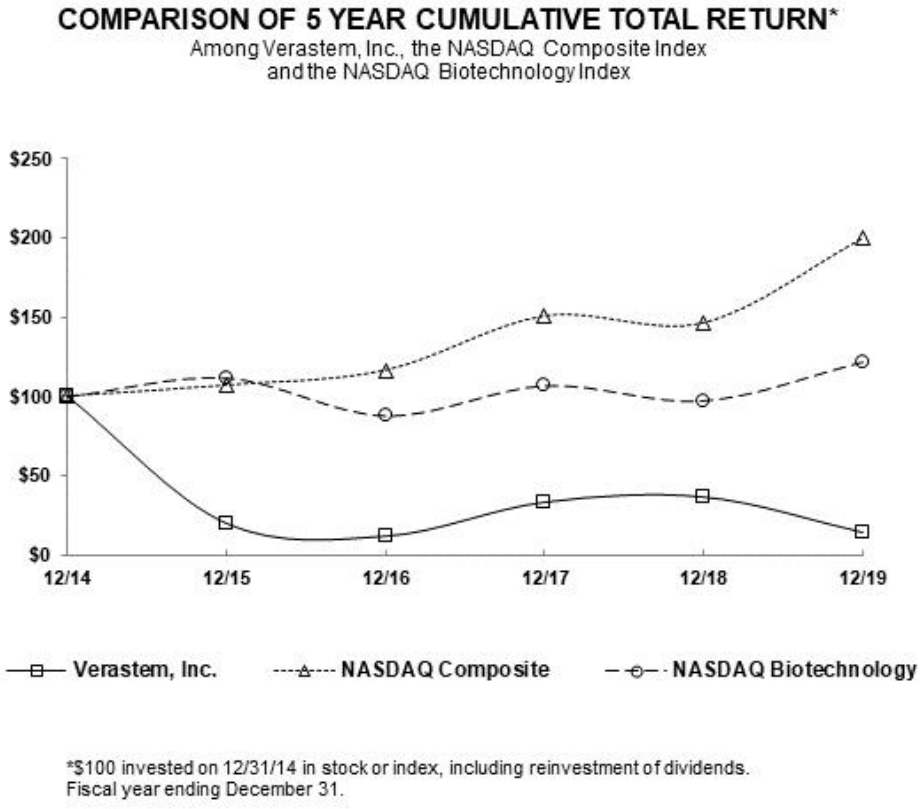
DIVIDENDS

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, 2014 through December 31, 2019. The comparison assumes $100 was invested after the market closed on December 31, 2014 in our common stock and in each of the foregoing indices, and it assumes reinvestment of dividends, if any.



* $100 invested on 12/31/14 in stock or index, including reinvestment of dividends. Fiscal year ending December 31, 2019.

Cumulative Total Return Comparison

	December 31,					
	2014	2015	2016	2017	2018	2019
Verastem, Inc.	100.00	20.35	12.25	33.59	36.76	14.66
NASDAQ Composite	100.00	105.73	113.66	145.76	140.10	189.45
NASDAQ Biotechnology	100.00	111.42	87.26	105.64	95.79	119.17

PURCHASE OF EQUITY SECURITIES

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

Item 6. Selected Financial Data

You should read the following selected financial data together with our consolidated financial statements and the related notes appearing elsewhere in this Annual Report on Form 10-K and the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section of this Annual Report on Form 10-K. The selected historical financial information in this section is not intended to replace our financial statements and the related notes therein. Our historical results for any prior period are not necessarily indicative of results to be expected in any future period.

	Year ended December 31,				
Statement of operations data:	**2019**	**2018**	**2017**	**2016**	**2015**
	(in thousands, except share and per share amounts)				
Revenue:					
Product revenue, net	$ 12,339	$ 1,718	$ —	$ —	$ —
License and collaboration revenue	5,117	25,000	—	—	—
Total revenue	17,456	26,718	—	—	—
Operating expenses:					
Cost of sales - product	$ 1,238	$ 165	$ —	$ —	$ —
Cost of sales - intangible amortization	1,569	423	—	—	—
Research and development	45,778	43,648	46,423	19,779	40,565
Selling, general and administrative	101,212	77,265	21,381	17,223	17,634
Total operating expenses	149,797	121,501	67,804	37,002	58,199
Loss from operations	(132,341)	(94,783)	(67,804)	(37,002)	(58,199)
Other (expense)/ income	(641)	25,556	—	—	—
Interest income	4,381	2,603	561	562	334
Interest expense	(20,608)	(5,810)	(559)	—	—
Net loss	$ (149,209)	$ (72,434)	$ (67,802)	$ (36,440)	$ (57,865)
Net loss per share—basic	$ (2.00)	$ (1.12)	$ (1.76)	$ (0.99)	$ (1.61)
Net loss per share—diluted	$ (2.00)	$ (1.37)	$ (1.76)	$ (0.99)	$ (1.61)
Weighted average common shares outstanding used in computing:					
Net loss per share—basic	74,578	64,962	38,422	36,988	35,932
Net loss per share—diluted	74,578	69,321	38,422	36,988	35,932

	As of December 31,				
Balance sheet data:	**2019**	**2018**	**2017**	**2016**	**2015**
	(in thousands)				
Cash, cash equivalents and investments	$ 75,506	$ 249,653	$ 86,672	$ 80,897	$ 110,258
Working capital	55,071	216,182	70,659	70,304	100,734
Total assets	145,046	277,236	89,791	83,629	113,094
Total debt	103,623	120,453	14,828	—	—
Accumulated deficit	(524,785)	(375,576)	(303,142)	(235,323)	(198,883)
Total stockholders' equity	7,174	124,299	57,684	72,297	102,469

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. Our marketed product, COPIKTRA® (duvelisib) capsules, and most advanced product candidates, defactinib and CH5126766 also referred to as VS-6766, utilize a multi-faceted approach to treat cancers originating either in the blood or major organ systems. We are currently developing duvelisib and our product candidates in both preclinical and clinical studies as potential therapies for certain cancers, including leukemia, lymphoma, head and neck cancer, ovarian cancer, colorectal cancer, lung cancer, pancreatic cancer, and mesothelioma. We believe that these compounds may be beneficial as therapeutics either as single agents or when used in combination with immuno-oncology agents, other pathway inhibitors or other current and emerging standard of care treatments in aggressive cancers that do not adequately respond to currently available therapies.

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 duvelisib and our product candidates and initiating U.S. commercial operations following the approval of COPIKTRA. 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 Sanofi, Yakult and CSPC, and the issuance of $150.0 million aggregate principal amount of 2018 Notes in October 2018. 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, 2019, we had an accumulated deficit of $524.8 million. Our net loss was $149.2 million, $72.4 million, and $67.8 million the years ended December 31, 2019, 2018 and 2017 respectively. We expect to incur significant expenses and 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, 2019, we had cash, cash equivalents, restricted cash and short-term investments of $111.3 million, inclusive of $35.7 million of restricted cash. On March 3, 2020, we received gross proceeds of $100.0 million from the sale of 46,511,628 shares of Common Stock. We expect our existing cash resources including proceeds from the sale of Common Stock in March 2020, along with revenue we expect to generate from sales of COPIKTRA, will be sufficient to fund our planned operations through 12 months from the date of issuance of these 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 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 and collaboration revenue to date has been generated through our license and collaboration agreements for the development and commercialization of duvelisib with Sanofi in the Sanofi Territory, 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 the Sanofi Territory, China or Japan.

Costs of sales - product

Cost of sales - product consist of costs of COPIKTRA on which product revenue was recognized, royalties owed to Healthcare Royalty Partners (HCR) and Infinity we incur as a result of such sales of COPIKTRA, and certain period costs. We expensed the manufacturing costs of COPIKTRA as operating 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 New Drug Application (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 2019 were expensed prior to the September 2018 FDA marketing approval and, therefore, are not included in cost of sales during this period. We expect cost of sales - product to increase in relation to product revenues as we deplete these inventories.

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.

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.

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, 2019, 2018 and 2017. 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 $11.3 million, $9.2 million and $5.8 million of personnel costs for the years ended December 31, 2019, 2018 and 2017, respectively.

	Year ended December 31,		
	2019	2018	2017
	(in thousands)	(in thousands)	(in thousands)
COPIKTRA	$ 25,518	$ 24,771	$ 30,409
Defactinib	1,823	2,230	2,894
Unallocated and other research and development expense	16,936	14,604	11,739
Unallocated stock-based compensation expense	1,501	2,043	1,381
Total research and development expense	$ 45,778	$ 43,648	$ 46,423

Our research and development expenses may 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.

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, legal, information technology, commercial, communication, human resources, and business development functions. Other selling, general and administrative expenses include allocated facility costs, commercial costs, 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 expense in 2019 consists entirely of the mark-to-market adjustment of the bifurcated make-whole interest provision derivative liability related to the 2019 Notes. Other income in 2018 consists entirely of the mark-to-market adjustment of the bifurcated conversion option derivative liability related to the 2018 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.

Revenue Recognition

The Company recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the Company expects to receive in exchange for those goods or services in accordance with ASC 606 *Revenue from Contracts with Customers*. 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, 2019, 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, 2019.

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, 2019.

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.

Subject to certain limitations, our 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.

As of December 31, 2019, we have not received any returns.

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, 2019.

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 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

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.

Stock-based compensation

We recognize stock-based compensation expense for stock options, and restricted stock units (RSUs) issued to employees and directors based on the grant date fair value of the awards on a straight-line basis over the requisite service period. Historically, we recorded stock-based compensation expense for stock options and RSUs 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. Effective January 1, 2019, we adopted Accounting Standard Updated (ASU) 2018-

09, *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. Upon adoption, we recognize stock-based compensation expense for stock options and RSUs issued to non-employees based on the grant date fair value of the awards on the straight-line basis over the requisite service period.

We estimate the fair value of stock option awards using the Black-Scholes option-pricing model. Determining the fair value of stock options 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 stock options 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 stock-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, 2019, 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 issue shares under the Company's employee stock purchase plan (ESPP) to employees. Stock-based compensation expense for discounted purchases under the ESPP is measured using the Black-Scholes model to compute the fair value of the lookback provision plus the purchase discount and is recognized as compensation expense over the offering period.

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. Stock-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, stock-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 stock-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 stock-based compensation expense could vary significantly from period to period.

As of December 31, 2019, there was approximately $14.0 million of unrecognized stock-based compensation related to stock options, which are expected to be recognized over a weighted-average period of 2.8 years. As of December 31, 2019, 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 2.6 years. See Notes 2 and 11 to our consolidated financial statements located in this Annual Report on Form 10-K for further discussion of stock-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, 2019.

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 sales-product. 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 sales - product in the consolidated statements of operations and comprehensive loss.

Shipping and handling costs for product shipments are recorded as incurred in costs of sales - product 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 or asset group 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.

Leases

Effective January 1, 2019, we adopted ASC 842. This standard 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.

At the inception of an arrangement, we determine whether the arrangement is or contains a lease based on the unique facts and circumstances within the arrangement. A lease is identified where an arrangement conveys the right to control the use of identified property, plant, and equipment for a period of time in exchange for consideration. Leases which are identified within the scope of ASC 842 and which have a term greater than one year are recognized on our consolidated balance sheets as right-of-use assets, lease liabilities and, if applicable, long-term lease liabilities. We have elected not to recognize leases with terms of one year or less on our consolidated balance sheets. Operating lease liabilities and their corresponding right-of-use assets are recorded based on the present value of lease payments over the expected remaining lease term. However, certain adjustments to the right-of-use asset may be required for items such as initial direct costs paid or incentives received. The interest rate implicit in lease contracts is typically not readily determinable. As a result, we utilize our incremental borrowing rates to calculate the present value of lease payments. Incremental borrowing rates are the rates we incur to borrow on a collateralized basis over a similar term an amount equal to the lease payments in a similar economic environment.

In accordance with ASC 842, components of a lease are split into three categories: lease components (e.g. land, building, etc.), non-lease components (e.g. common area maintenance, maintenance, consumables, etc.), and non-components (e.g. property taxes, insurance, etc.). The fixed and in-substance fixed contract consideration (including any related to non-components) must be allocated based on fair values to the lease components and non-lease components. Although separation of lease and non-lease components is required, certain practical expedients are available. Entities may elect the practical expedient to not separate lease and non-lease components. Rather, they would account for each lease component and the related non-lease component together as a single component. We have elected to account for the lease and non-lease components of each of our operating leases as a single lease component and allocate all of the contract consideration to the lease component only. The lease component results in an operating right-of-use asset being recorded on the consolidated balance sheets and amortized on a straight-line basis as lease expense.

RESULTS OF OPERATIONS

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

	Year Ended December 31,		
	2019	2018	2017
Revenue:			
Product revenue, net	12,339	1,718	—
License and collaboration revenue	5,117	25,000	—
Total revenue	17,456	26,718	—
Operating expenses:			
Cost of sales - product	$ 1,238	$ 165	$ —
Cost of sales - intangible amortization	1,569	423	—
Research and development	45,778	43,648	46,423
Selling, general and administrative	101,212	77,265	21,381
Total operating expenses	149,797	121,501	67,804
Loss from operations	(132,341)	(94,783)	(67,804)
Other (expense)/ income	(641)	25,556	—
Interest income	4,381	2,603	561
Interest expense	(20,608)	(5,810)	(559)
Net loss	$ (149,209)	$ (72,434)	$ (67,802)

Comparison of the Year Ended December 31, 2019 to the Year Ended December 31, 2018

Product revenue, net. Product revenue net for the year ended December 31, 2019 (2019 Period) was $12.3 million compared to $1.7 million for the year ended December 31, 2018 (2018 Period). Product revenue, net consisted of net product sales of COPIKTRA in the United States. We began commercial sales of COPIKTRA within the United States in September 2018 following receipt of FDA marketing approval. The $10.6 million increase was driven primarily by an increase in product shipments for COPIKTRA as a result of greater market penetration.

License and collaboration revenue. License and collaboration revenue for the 2019 Period was $5.1 million compared to $25.0 million for the 2018 Period. The $19.9 million decrease was related to a $10.0 million upfront payment received in connection to our license and collaboration agreement with Yakult and a $15.0 million upfront payment received in connection to our license and collaboration agreement with CSPC in the 2018 period, partially offset by a $5.0 million upfront payment received in connection to our collaboration agreement with Sanofi and collaboration revenue of $0.1 million related to the shipment of clinical supply of COPIKTRA to Yakult and CSPC during the 2019 Period.

Costs of sales – product. Costs of sales – product for the 2019 Period was $1.2 million compared to $0.2 million for the 2018 period. The $1.0 million increase was primarily driven by an increase in the volume of COPIKTRA sold and corresponding increases in royalties, manufacturing and other costs during the 2019 Period as compared to the 2018 Period. Cost of sales - product consisted of costs associated with the manufacturing of COPIKTRA, royalties owed to HCR and Infinity on such sales, and certain period costs. We expensed the manufacturing costs of COPIKTRA as operating 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 New Drug Application (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 2019 Period were expensed prior to the September 2018 FDA marketing approval and, therefore, are not included in cost of sales during this period. We expect cost of sales - product to increase in relation to product revenues as we deplete these inventories. Our inventory balance as of December 31, 2019 has increased compared to December 31, 2018 due to manufacturing of COPIKTRA in 2019.

Research and development expense. Research and development expense for the 2019 Period was $45.8 million compared to $43.6 million for the 2018 Period. The $2.2 million increase from the 2018 Period to the 2019 Period was primarily related to an increase of $3.7 million of contract research organization (CRO) costs for our Phase 2 Intermittent Dosing study entitled TEMPO, increase of $1.3 million for CRO costs related to our Phase 2 study for the treatment of PTCL – entitled PRIMO, an increase of $1.6 million in personnel related costs, including non-cash stock-based compensation and $0.5 million of other costs. This increase is partially offset by a decrease of $2.4 million in consulting fees as a result of activities to file an NDA in the 2018 Period and a decrease of $2.5 million in CRO costs as a result of site closures in our Phase 3 DUO and Phase 2 DYNAMO studies throughout 2018 and 2019 as patients continue to complete treatment.

Selling, general and administrative expense. Selling, general and administrative expense for the 2019 Period was $101.2 million compared to $77.3 million for the 2018 Period. The increase of $23.9 million from the 2018 Period to the 2019 Period primarily resulted from an increase in personnel related costs, including non-cash stock-based compensation, of $14.2 million, primarily related to the hiring and staffing of our sales and commercial teams, an increase in consulting and professional fees of $6.7 million, primarily related to the support of the commercial launch activities, and an increase in travel and other costs of $3.0 million.

Cost of Sales – intangible amortization. Cost of sales – intangible amortization for the 2019 Period was approximately $1.6 million compared to $0.4 million for the 2018 Period. Cost of sales – intangible amortization was related to the COPIKTRA finite-lived intangible asset which we recognized and began amortizing in September 2018.

Other (expense)/ income. Other expense for the 2019 Period of approximately $0.6 million was for the mark-to-market adjustment related to the bifurcated make-whole interest provision derivative liability related to the 2019 Notes. 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 2018 Notes.

Interest income. Interest income for the 2019 Period was $4.4 million compared to $2.6 million for the 2018 Period. The increase of $1.8 million from the 2018 Period to the 2019 period is primarily due to higher investment cost basis and higher interest rates on investments.

Interest expense. Interest expense for the 2019 Period was $20.6 million compared to $5.8 million for the 2018 Period. The increase of $14.8 million was due to the issuance of the 2018 Notes in October 2018, a higher principal balance and higher interest rates on our loan and security agreement with Hercules and the acceleration of an end of term fee related to the Hercules loan and security agreement refinancing recorded as interest expense.

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 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 and collaboration revenue. License and collaboration 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 and collaboration revenue during the 2017 Period.

Costs of sales – product. Costs of sales - product 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 had no cost of sales - product 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.

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.

Cost of Sales – intangible amortization. Cost of sales – intangible amortization 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 cost of sales – intangible amortization in the 2017 Period.

Other income/(expense) 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.

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 Sanofi, 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, 2019, we had $111.3 million in cash, cash equivalents, restricted cash and short-term investments inclusive of $35.7 million in restricted cash. 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,		
	2019	2018	2017
Net cash (used in) provided by:			
Operating activities	$ (138,518)	$ (74,515)	$ (57,310)
Investing activities	89,613	(138,377)	43,953
Financing activities	(2,441)	261,162	63,184
Increase (decrease) in cash, cash equivalents and restricted cash	**$ (51,346)**	$ 48,270	$ 49,827

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 $64.0 million increase in cash used in operating activities for the 2019 Period compared to the 2018 Period was primarily due to an increase in selling, general, and administrative expenses related to the post-launch commercial operations supporting COPIKTRA, and due to a $10.0 million license payment from Yakult and $15.0 million license payment received from CSPC during the 2018 Period, partially offset by a $5.0 million license payment received from Sanofi during the 2019 Period. The $17.2 million increase in cash used in operating activities for the 2018 Period compared to the 2017 Period 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.

Investing activities. The cash provided by investing activities for the 2019 Period relates to the net maturities of investments of $89.6 million. 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.

Financing activities. The cash used by financing activities for the 2019 Period represents $12.2 million principal payments on the 2018 Notes, $0.4 million of interest-make whole payments on the 2019 Notes and $0.1 million of payments for settlement of restricted stock for tax withholdings. This is partially offset by $9.7 million of net proceeds as a result of the Amendment to the loan and security agreement with Hercules and $0.6 million of proceeds received related to exercise of stock option and employee stock purchase plan. The cash provided by financing activities for the 2018 Period primarily represents $145.3 million in net proceeds received from the issuance of 2018 Notes, $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.

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 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. The Original Loan Agreement was amended on January 4, 2018, March 6, 2018, and October 11, 2018 (the Amended Loan Agreement) to increase the total borrowing limit under the Original Loan Agreement from $25.0 million up to $50.0 million (the Amended Term Loan), pursuant to certain conditions of funding.

On April 23, 2019 (the Fourth Amendment Date) and November 14, 2019 (the Fifth Amendment Date), we entered into the Fourth Amendment and Fifth Amendment (together the Amendments) to the Original Loan Agreement with Hercules. The Amendments further amend the Amended Loan Agreement (together, with the Amendments, the 2019 Term Loan Agreement).

Per the terms of the 2019 Term Loan Agreement, we may borrow up to an aggregate of $75.0 million, of which $35.0 million was outstanding immediately as of the Fourth Amendment Date (2019 Term A Loan) as a result of the existing outstanding principal of term loans of $25.0 million under the Amended Loan Agreement being converted into the 2019 Term A Loan, and an additional $10.0 million being drawn on the Fourth Amendment Date. The remaining $40.0 million of borrowing capacity may be drawn in multiple tranches comprised of (i) a term loan in an amount of up to $15.0 million upon us generating cumulative net product revenues (as defined in the 2019 Term Loan Agreement) of either (a) $37.5 million on or before April 30, 2020 or (b) $50.0 million on or before June 30, 2020 (2019 Term B Loan), and (ii) a term loan in an amount of up to $25.0 million available through December 31, 2021, subject to Hercules' approval and certain other conditions specified in the 2019 Term Loan Agreement (the 2019 Term C Loan, and together with the 2019 Term A Loan and 2019 Term B Loan, the 2019 Term Loan). As of December 31, 2019, we have borrowed a total of $35.0 million in term loans.

The 2019 Term Loan will mature on December 1, 2022 (2019 Term Loan Maturity Date). Each advance accrues interest at a floating per annum rate equal to the greater of (a) 9.75% or (b) the lesser of (i) 12.00% and (ii) the sum of (x) 9.75% plus (y) (A) the prime rate minus (B) 5.50%. The 2019 Term Loan provides for interest-only payments until April 1, 2021, which may be extended to December 1, 2021 pursuant to us generating $40.0 million in net product revenue on a trailing six-month basis on or prior to December 31, 2020 provided that no event of default has occurred. Thereafter, amortization payments will be payable monthly in equal installments of principal and interest (subject to recalculation upon a change in prime rates).

The 2019 Term Loan is secured by a lien on substantially all of our assets, other than intellectual property and contains customary covenants and representations, including a liquidity covenant, minimum net revenue covenant, financial reporting covenant and limitations on dividends, indebtedness, collateral, investments, distributions, transfers, mergers or acquisitions, taxes, corporate changes, deposit accounts, and subsidiaries.

On the Fourth Amendment Date, we were required to pay any outstanding accrued interest as well as the final payment fee equal to 4.5% on the outstanding principal balance of the Amended Term Loan, or $1.1 million. No prepayment charges were due as a result of executing the Amendment or conversion of the existing term loans into 2019 Term A Loans.

The events of default under the 2019 Term 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 2019 Term Loan Agreement, promissory notes or other loan documents, (ii) any breach or default in the performance of any covenant under the 2019 Term 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 2019 Term Loan Agreement.

The 2019 Term Loan Agreement also contains other customary provisions, such as expense reimbursement and confidentiality. Hercules has indemnification rights and the right to assign the 2019 Term Loan.

On March 30, 2017, we established an at-the-market equity offering program (ATM) pursuant to which 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 Fitzgerald & Co. (Cantor), as sales agent. 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 ATM to $75.0 million. Through December 31, 2018, we sold 11,518,354 shares under the ATM for net proceeds of approximately $47.3 million (after deducting commissions and other offering expenses). During the 2019 Period, there were no sales under the ATM. As of December 31, 2019, we can issue an addition $26.6 million of gross proceeds under this program.

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 2018 Notes) , for net proceeds of approximately $145.3 million. The 2018 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 2018 Indenture), each dated October 17, 2018, by and between us and Wilmington Trust, National Association, as trustee. The 2018 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 2018 Notes will mature on November 1, 2048, unless earlier repurchased, redeemed or converted in accordance with their terms.

The 2018 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 2018 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 2018 Notes then outstanding to be converted automatically if the “Daily VWAP” (as defined in the 2018 Indenture) per share of our common stock equals or

exceeds 130% of the conversion price, which equates to approximately $9.31 per share, 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 2018 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 2018 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 2018 Notes, including the conversion, put and call features. Per the terms of the 2018 Indenture, upon conversion of the 2018 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 conversion feature derivative was determined based on the difference between the fair value of the 2018 Notes with the conversion option and the fair value of the 2018 Notes without the conversion option. We determined that the fair value of the derivative upon issuance of the 2018 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 2018 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. Accordingly, the conversion feature will no longer be measured at fair value on our financial statements.

On November 14, 2019 and December 23, 2019, we entered into privately negotiated agreements to exchange approximately $114.3 million and $7.4 million, respectively, aggregate principal amount of the 2018 Notes for (i) approximately $62.9 million and $4.0 million, respectively, aggregate principal amount of 5.00% Convertible Senior Second Lien Notes due 2048 (the 2019 Notes) (ii) an aggregate of approximately $11.4 million and $0.7 million in 2018 Notes principal repayment and (iii) accrued interest on the 2018 Notes through November 14, 2019 and December 23, 2019, respectively. The 2019 Notes are governed by the terms of an indenture (the 2019 Indenture). The 2019 Notes are senior secured obligations of the Company and bear interest at 5.00% per annum, payable semi-annually in arrears on May 1 and November 1 of each year. The 2019 Notes will mature on November 1, 2048, unless earlier repurchased, redeemed or converted in accordance with the terms.

The 2019 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 606.0606 shares of common stock per $1,000 principal amount of the 2019 Notes, which corresponds to an initial conversion price of approximately $1.65 per share of common stock and represents a conversion premium of approximately 52.8% above the last reported sale price of our common stock of $1.08 per share on November 11, 2019.

We will have the right, exercisable at our option, to cause all 2019 Notes then outstanding to be converted automatically if the “Daily VWAP” (as defined in the 2019 Indenture) per share of our common stock equals or exceeds 121% of the conversion price, which equates to approximately $2.00 per share, 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 2019 Notes. (Company’s Mandatory Conversion Option)

Upon conversion, converting noteholders will be entitled to receive accrued interest on their converted 2019 Notes. In addition, if the 2019 Notes are converted with a conversion date that is on or prior to November 1, 2020, other than in connection with the Company’s exercise of the Company’s Mandatory Conversion Option then

the consideration due upon any such conversion will also include a cash interest make-whole payment for all future scheduled interest payments on the converted 2019 Notes through November 1, 2020 (2019 Notes Interest Make-Whole Provision).

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 2019 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 2019 Notes, including the conversion, put and call features. In consideration of the 2019 Notes Interest Make-Whole Provision, we concluded the provision required bifurcation as a derivative. The fair value of the 2019 Interest Make-Whole Provision was determined using a Monte Carlo model. It was determined that the fair value of the derivative upon the November 14, 2019 and December 23, 2019 issuance of the 2019 Notes was an aggregate of $0.2 million, and we recorded this amount as a derivative liability and the offsetting amount as a debt discount as a reduction to the carrying value of the 2019 Notes on the closing dates. During the period November 14, 2019 to December 31, 2019, we paid out approximately $0.4 million in 2019 Interest Make-Whole payments which was recorded as a reduction of the derivative liability. As of December 31, 2019, we determined the fair value of the 2019 Interest Make-Whole Provision was $0.5 million. We recorded the change in the fair value of the 2019 Interest Make-Whole Provision for the period from November 14, 2019 to December 31, 2019 of $0.6 million as other expense on the consolidated statements of operations and comprehensive loss.

As of December 31, 2019, there was $28.3 million and $57.4 million aggregate principal amount outstanding of the 2018 Notes and 2019 Notes, respectively, for a total of $85.7 million aggregate principal amount outstanding compared to $150.0 million aggregate principal amount outstanding of 2018 Notes as of December 31, 2018. During the 2019 Period, 2019 Note holders converted $9.5 million aggregate principal of 2019 Notes in exchange for 5,767,872 shares of common stock and $0.4 million of cash for 2019 Interest Make-Whole Provision.

Funding requirements

We expect to continue to incur significant expenses and operating losses. We anticipate that our expenses and operating losses will continue as we:

- commercialize COPIKTRA;
- continue our ongoing clinical trials, including with COPIKTRA, defactinib and CH5126766;
- 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 resources, including proceeds from the sale of Common Stock in March 2020 along with the revenue we expect to generate from COPIKTRA 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. 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.

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, 2019:

(in thousands)	Total	2020	2021 - 2022	2023 - 2024	Thereafter
Operating lease obligations	$ 5,700	$ 955	$ 2,058	$ 2,141	$ 546
2019 Term Loan Agreement	35,000	—	35,000	—	—
2018 Notes	28,300	—	—	—	28,300
2019 Notes	57,414	—	—	—	57,414
License agreements (1)	—	—	—	—	—

(1) As discussed in Note 16 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, 2019, we had federal and state net operating loss carryforwards of $371.7 million and $392.3 million, respectively, which are available to reduce future taxable income. We also had federal and state tax credits of $20.6 million and $3.0 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 2039, except for $136.3 million of federal net operating loss carryforwards which may be carried forward indefinitely. 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, 2019, we recorded a 100% valuation allowance against our net operating loss and tax credit carryforwards of $128.4 million, as we believe it is more likely than not that the tax benefits will not be fully realized. In the future, if we determine that a portion or all of the tax benefits associated with our tax carryforwards will be realized, net income would increase in the period of determination.

RECENTLY ADOPTED ACCOUNTING STANDARDS

In 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 was 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 adopted this standard prospectively effective January 1, 2019. The adoption of this ASU did not have an effect on the Company's consolidated financial statements or 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* (ASC 842). 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 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 adopted this standard using the optional transition method effective January 1, 2019. Upon adoption of this standard, the Company recognized a lease liability and a corresponding right-of use asset of $4.0 million and $3.4 million, respectively, and derecognized a deferred rent liability and a corresponding lease incentive obligation of $0.4 million and $0.2 million, respectively. The Company did not record any cumulative effect adjustment to accumulated deficit as a result of adopting this standard. The Company also elected to adopt the practical expedients upon transition, which permit companies to not reassess lease identification, classification, and initial direct costs under ASU 2016-02 for leases that commenced prior to the effective date.

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, restricted cash and short-term investments of $111.3 million and $250.4 million as of December 31, 2019 and 2018, respectively, inclusive of 35.7 million and $0.7 million of restricted cash as of December 31, 2019 and 2018, 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, 2019, an immaterial amount of our total liabilities was denominated in currencies other than the functional currency.

As of December 31, 2019, we have borrowed $35.0 million under the 2019 Term Loan Agreement. The 2019 Term Loan Agreement bears interest per annum equal to the greater of either (a) 9.75% or (b) the lesser of (i) 12.00% and (ii) the sum of (x) 9.75% plus (y) (A) the prime rate minus (B) 5.50%. Changes in interest rates can cause interest charges to fluctuate under the 2019 Term 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, 2019.

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 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 Business and 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 Business and 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 Business and 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 Business and 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, 2019.

The effectiveness of our internal control over financial reporting as of December 31, 2019 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, 2019 that has materially affected, or is reasonably likely to materially affect, the Company's internal control over financial reporting.

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, 2019, 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, 2019, 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, 2019 and 2018, 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, 2019, and the related notes of the Company and our report dated March 11, 2020 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 11, 2020

Item 9B. Other Information

None.

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 2020 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 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 2020 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 2020 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 2020 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 2020 Proxy Statement and is incorporated herein by reference.

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.

EXHIBIT INDEX

Exhibit number	Description of exhibit
3.1	Restated Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.1 to the Annual Report on Form 10-K filed by the Registrant on March 12, 2019)
3.2	Certificate of Amendment to the Restated Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.2 to the Annual Report on Form 10-K filed by the Registrant on March 12, 2019)
3.3	Amended and Restated Bylaws of the Registrant (incorporated by reference to Exhibit 3.4 to Amendment No. 3 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on January 13, 2012)
4.1	Specimen certificate evidencing shares of common stock (incorporated by reference to Exhibit 4.1 to Amendment No. 3 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on January 13, 2012)
4.2	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)
4.3	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)
4.4	Indenture, dated as of November 14, 2019, between Verastem, Inc. and Wilmington Trust, National Association(incorporated by reference to Exhibit 4.1 to the Current Report on Form 8-K filed by the Registrant on November 20, 2019)
4.5	Description of Securities
10.1#	2010 Equity Incentive Plan (incorporated by reference to Exhibit 10.1 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on November 3, 2011)
10.2#	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)
10.3#	Form of Incentive Stock Option Agreement under 2012 Incentive Plan (incorporated by reference to Exhibit 10.3 to Amendment No. 3 to the Registration Statement on Form S-1 (File No. 333-177677) filed by the Registrant on January 13, 2012)
10.4#	Form of 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)
10.5#	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)
10.6#	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)

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)
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#	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)
10.15	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)
10.16	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)
10.17#	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)
10.18†	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)
10.19†	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)
10.20#	Amended and Restated Employment Agreement, dated November 22, 2013, by and between the Registrant and Robert Forrester (incorporated by reference to Exhibit 10.32 to the Annual Report on Form 10-K filed by the Registrant on March 6, 2014)
10.21#	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)

10.22†	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)
10.23	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)
10.24	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)
10.25	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)
10.26	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)
10.27#	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)
10.28†	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)
10.29†	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)
10.30	Employment Agreement between the Registrant and Brian Stuglik, dated July 29, 2019 (incorporated by reference to Exhibit 10.1 to Current Report on Form 8-K filed by the Registrant on August 1, 2019)
10.31	Fourth Amendment to the 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 Current Report on Form 8-K filed by the Registrant on April 23, 2019)
10.32	Fifth Amendment to the 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 Current Report on Form 8-K filed by the Registrant on November 20, 2019)
10.33*	Consulting Agreement, dated June 21, 2019, between Robert Forrester and Verastem, Inc.
10.34*	Separation Agreement, dated June 25, 2019, between Robert Forrester and Verastem, Inc.
10.35‡	License and Collaboration Agreement, dated July 25, 2019, between Verastem, Inc. and Sanofi (incorporated by reference to Exhibit 10.1 to Quarterly Report on Form 10-Q filed by the Registrant on October 30, 2019)
10.36	Purchase Agreement, dated February 27, among Verastem, Inc. and each purchaser party thereto (incorporated by reference to Exhibit 10.1 to Current Report on Form 8-K filed by the Registrant on February 28, 2020)

10.37*	Consulting Agreement, dated June 27, 2019, between Joseph Lobacki and Verastem, Inc.
21.1*	Subsidiaries of the Registrant
23.1*	Consent of Ernst & Young LLP
31.1*	Certification of the Chief Executive Officer pursuant to Exchange Act Rule 13a-14(a)
31.2*	Certification of the Chief Financial Officer pursuant to Exchange Act Rule 13a-14(a)
32.1*	Certification of the Chief Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
32.2*	Certification of the Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
99.1*	Press Release issued by Verastem, Inc. on March 11, 2020 (furnished herewith)
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

* Filed herewith.

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

‡ Certain confidential information contained in this exhibit has been omitted because it (i) is not material and (ii) would be competitively harmful if publicly disclosed.

Management contract or compensatory plan, contract or agreement.

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 11th day of March 2020.

VERASTEM, INC.
By: /s/ Brian M. stuglik

Brian M. Stuglik
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/ Brian M. Stuglik R.Ph Brian M. Stuglik	Chief Executive Officer and Director (Principal executive officer)	March 11, 2020
/s/ Robert Gagnon Robert Gagnon	Chief Business and Financial Officer (Principal financial and accounting officer)	March 11, 2020
/s/ Timothy Barberich Timothy Barberich	Director	March 11, 2020
/s/ Gina Consylman Gina Consylman	Director	March 11, 2020
/s/ Michael Kauffman, M.D.,Ph.D. Michael Kauffman, M.D., Ph.D.	Director	March 11, 2020
/s/ Alison Lawton Alison Lawton	Director	March 11, 2020
/s/ Eric Rowinsky, M.D. Eric Rowinsky, M.D.	Director	March 11, 2020
/s/ Bruce Wendel Bruce Wendel	Director	March 11, 2020

Verastem, Inc.

CONSOLIDATED FINANCIAL STATEMENTS

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, 2019 and 2018, 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, 2019, 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, 2019 and 2018, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2019, 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, 2019, 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 11, 2020 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 11, 2020

Verastem, Inc.
CONSOLIDATED BALANCE SHEETS
(in thousands, except per share amounts)

	December 31, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 43,514	$ 129,867
Short-term investments	31,992	119,786
Accounts receivable, net	2,524	306
Inventory	3,096	327
Prepaid expenses and other current assets	3,835	2,973
Total current assets	84,961	253,259
Property and equipment, net	947	1,369
Right-of-use asset, net	3,077	—
Intangible assets, net	20,008	21,577
Restricted Cash	35,241	241
Other assets	812	790
Total assets	$ 145,046	$ 277,236
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 9,655	$ 10,253
Accrued expenses	19,365	21,108
Lease liability, short-term	420	—
Derivative liability, short-term	450	—
Current portion of long-term debt	—	5,716
Total current liabilities	29,890	37,077
Non-current liabilities:		
Long-term debt	35,067	19,506
Convertible senior notes	68,556	95,231
Lease liability, long-term	3,489	—
Other non-current liabilities	870	1,123
Total liabilities	137,872	152,937
Stockholders' equity:		
Preferred stock, $0.0001 par value; 5,000 shares authorized, no shares issued and outstanding at December 31, 2019 and December 31, 2018, respectively	—	—
Common stock, $0.0001 par value; 200,000 shares authorized, 80,118 and 73,806 shares issued and outstanding at December 31, 2019 and December 31, 2018, respectively	8	7
Additional paid-in capital	531,937	499,741
Accumulated other comprehensive income	14	127
Accumulated deficit	(524,785)	(375,576)
Total stockholders' equity	7,174	124,299
Total liabilities and stockholders' equity	$ 145,046	$ 277,236

See accompanying notes to the consolidated financial statements.

Verastem, Inc.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(in thousands, except per share amounts)

	Year Ended December 31,		
	2019	2018	2017
Revenue:			
Product revenue, net	$ 12,339	$ 1,718	$ —
License and collaboration revenue	5,117	25,000	—
Total revenue	17,456	26,718	—
Operating expenses:			
Cost of sales - product	$ 1,238	$ 165	$ —
Cost of sales - intangible amortization	1,569	423	—
Research and development	45,778	43,648	46,423
Selling, general and administrative	101,212	77,265	21,381
Total operating expenses	149,797	121,501	67,804
Loss from operations	(132,341)	(94,783)	(67,804)
Other (expense)/ income	(641)	25,556	—
Interest income	4,381	2,603	561
Interest expense	(20,608)	(5,810)	(559)
Net loss	$ (149,209)	$ (72,434)	$ (67,802)
Net loss per share—basic	$ (2.00)	$ (1.12)	$ (1.76)
Net loss per share—diluted	$ (2.00)	$ (1.37)	$ (1.76)
Weighted average common shares outstanding used in computing:			
Net loss per share—basic	74,578	64,962	38,422
Net loss per share—diluted	74,578	69,321	38,422
Net loss	$ (149,209)	$ (72,434)	$ (67,802)
Unrealized (loss) gain on available-for-sale securities	(113)	129	(31)
Comprehensive loss	$ (149,322)	$ (72,305)	$ (67,833)

See accompanying notes to the consolidated financial statements.

Verastem, Inc.
CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(in thousands, except share data)

	Common stock		Additional paid-in capital	Accumulated other comprehensive (loss) income	Accumulated deficit	Total stockholders' equity
	Shares	Amount				
Balance at December 31, 2016	**36,992,418**	**$ 4**	**$ 307,587**	**$ 29**	**$ (235,323)**	**$ 72,297**
Net loss	—	—	—	—	(67,802)	(67,802)
Unrealized (loss) on available-for-sale marketable securities	—	—	—	(31)	—	(31)
Issuance of common stock resulting from follow-on offering, net of issuance costs of $324	8,422,877	1	24,691	—	—	24,692
Issuance of common stock resulting from at-the-market transactions, net of issuance costs of $112	5,036,879	—	23,053	—	—	23,053
Issuance of common stock resulting from exercise of stock options	348,734	—	442	—	—	442
Stock-based compensation expense	—	—	5,050	—	(17)	5,033
Balance at December 31, 2017	**50,800,908**	**$ 5**	**$ 360,823**	**$ (2)**	**$ (303,142)**	**$ 57,684**
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**
Net loss	—	—	—	—	(149,209)	(149,209)
Unrealized loss on available-for-sale marketable securities	—	—	—	(113)	—	(113)
Conversion of Notes into common stock	5,767,872	1	9,516	—	—	9,517
Change in fair value of conversion option of Notes on exchange	—	—	13,640	—	—	13,640
Issuance of common stock under Employee Stock Purchase Plan	341,701	—	439	—	—	439
Issuance of common stock resulting from vesting of restricted stock units	109,707	—	(68)	—	—	(68)
Issuance of common stock resulting from exercise of stock options	91,907	—	130	—	—	130
Stock-based compensation expense	—	—	8,539	—	—	8,539
Balance at December 31, 2019	**80,117,531**	**$ 8**	**$ 531,937**	**$ 14**	**$ (524,785)**	**$ 7,174**

See accompanying notes to the consolidated financial statements.

Verastem, Inc.
CONSOLIDATED STATEMENTS OF CASH FLOWS
(in thousands)

	Year Ended December 31,		
	2019	2018	2017
Operating activities			
Net loss	$ (149,209)	$ (72,434)	$ (67,802)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation	429	996	556
Amortization of acquired intangible asset	1,569	423	—
Amortization of right-of-use asset and lease liability	181	—	—
Stock-based compensation expense	8,539	6,671	5,033
Amortization of deferred financing costs, debt discounts and premiums and discounts on available-for-sale marketable securities	7,131	1,814	223
Change in fair value of interest make whole provision and conversion option for Notes	641	(25,556)	—
Gain on sale of fixed assets	—	(79)	—
Changes in operating assets and liabilities:			
Accounts receivable, net	(2,218)	(306)	—
Inventory	(2,769)	(327)	—
Prepaid expenses, other current assets and other assets	(877)	(1,167)	(943)
Accounts payable	(598)	1,048	5,046
Accrued expenses and other liabilities	(1,707)	13,902	577
Other long-term liabilities	370	500	—
Net cash used in operating activities	(138,518)	(74,515)	(57,310)
Investing activities			
Purchases of property and equipment	(7)	(1,507)	—
Sales of property and equipment	—	82	—
Acquisition of intangible asset	—	(22,000)	—
Purchases of investments	(94,123)	(125,452)	(7,957)
Maturities of investments	183,743	10,500	51,910
Net cash provided by (used in) investing activities	89,613	(138,377)	43,953
Financing activities			
Proceeds from long-term debt, net of issuance costs	9,670	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 and employee stock purchase program	569	809	442
Principal payments on the convertible senior notes	(12,174)	—	—
Interest make-whole payments on the 2019 Notes	(438)	—	—
Settlement of restricted stock for tax withholdings	(68)	—	—
Proceeds from the issuance of common stock, net	—	105,156	48,069
Net cash (used in) provided by financing activities	(2,441)	261,162	63,184
(Decrease) increase in cash, cash equivalents and restricted cash	(51,346)	48,270	49,827
Cash, cash equivalents and restricted cash at beginning of period	130,608	82,338	32,511
Cash, cash equivalents and restricted cash at end of period	$ 79,262	$ 130,608	$ 82,338
Supplemental disclosure			
Cash paid for interest	$ 12,424	$ 2,107	$ 295
Supplemental disclosure of non-cash investing and financing activities			
Common stock issuance costs included in accounts payable and accrued expenses	$ 15	$ 15	$ 324
Change in fair value of conversion option of Notes on exchange	$ 13,640	$ —	$ —
Conversion of Notes into common stock	$ 9,517	$ —	$ —

See accompanying notes to the consolidated financial statements.

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 adult patients with certain hematologic cancers including 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. Its marketed product, COPIKTRA, and most advanced product candidates, defactinib and CH5126766, 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, head and neck cancer, ovarian cancer, colorectal cancer, lung cancer, pancreatic cancer, and mesothelioma. The Company believes that these compounds may be beneficial as therapeutics either as single agents or when used in combination with immuno-oncology agents, other pathway inhibitors or other current and emerging standard of care treatments in aggressive cancers that do not adequately respond to currently available therapies.

The consolidated financial statements include the accounts of Verastem Securities Company and Verastem Europe GmbH, wholly-owned subsidiaries of the Company. All financial information presented has been consolidated and includes the accounts of the Company and its wholly-owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation.

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 commercial success of COPIKTRA, or any of the Company's investigational product candidates following receipt of regulatory approval, protection of proprietary technology and the continued ability to obtain adequate financing to fund the Company's future operations. 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 as it continues the research and development of its product candidates and commercialization of COPIKTRA. As of December 31, 2019, the Company had cash, cash equivalents, restricted cash and short-term investments of $111.3 million, inclusive of $35.7 million of restricted cash, and accumulated deficit of $524.8 million. On March 3, 2020, the Company received gross proceeds of approximately $100.0 million from the sale of 46,511,628 shares of Common Stock (see Note 19). The Company expects its existing cash resources, including the proceeds from the sale of Common Stock in March 2020, along with revenue the Company expects to generate from sales of COPIKTRA, will be sufficient to fund its planned operations through 12 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.

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, 2019	December 31, 2018
Cash and cash equivalents	$ 43,514	$ 129,867
Restricted cash	35,748	741
Total cash, cash equivalents and restricted cash	**$ 79,262**	**$ 130,608**

Amounts included in restricted cash as of December 31, 2019 represent cash that the Company is contractually obligated to maintain in accordance with the terms of the 2019 Term Loan Agreement, cash received pursuant to a funded research and development agreement with the Leukemia and Lymphoma Society (LLS) (the "LLS Research Funding Agreement") 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 $35.0 million, $0.5 million, and $0.2 million respectively. Restricted cash related to 2019 Term Loan Agreement and letters of credit are included in non-current restricted cash on the consolidated balance sheet, while cash related to LLS Research Funding Agreement is included in prepaid and other current assets on the consolidated balance sheet. Amounts included in restricted cash as of December 31, 2018 represent cash received pursuant to the LLS Research Funding Agreement of $0.5 million, which is included in prepaid and other currents on the consolidated balance sheet and cash held to collateralize outstanding letters of credit provided as a security deposit for the Company's office space located in Needham of $0.2 million, which is included in non-current restricted cash on the consolidated balance sheet.

Fair value of financial instruments

The Company determines the fair value of its financial instruments based upon the fair value hierarchy, which prioritizes valuation inputs based on the observable nature of those inputs. The fair value hierarchy applies

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

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

Items Measured at Fair Value on a Recurring Basis

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

	December 31, 2019			
Description	**Total**	**Level 1**	**Level 2**	**Level 3**
Financial assets				
Cash equivalents	$ 77,176	$ 75,678	$ 1,498	$ —
Short-term investments	31,992	—	31,992	—
Total financial assets	**$ 109,168**	**$ 75,678**	**$ 33,490**	**$ —**
Derivative liability	$ 450	—	—	$ 450

	December 31, 2018			
Description	**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**	**$ —**

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, 2019 and 2018.

During 2019, a derivative liability was recorded as a result of the issuance of the 2019 Notes. (see note 12). The Company initially determined fair value of the liability upon issuance, and then again at the balance sheet date. The fair value measurement of the derivative liability is classified as Level 3 under the fair value hierarchy and it has been valued using unobservable inputs. These inputs include: (1) a simulated share price at the time of conversion of the 2019 Notes, (2) assumed timing of conversion of the 2019 Notes, (3) risk-adjusted discount rate to present value the probability-weighted cash flows, and (4) entity specific cost of equity. 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 Monte-Carlo simulation by calculating fair value of the 2019 Interest Make-Whole Payment to 2019 Note holders based on assumed timing of conversion of the 2019 Notes. At November 14, 2019 the risk-adjusted discount rate was determined to be 12.06% and entity specific

cost of equity was determined to be 17.05%. At December 31, 2019, the risk-adjusted discount rate was determined to be 13.08% and entity specific cost of equity was determined to be 16.54%.

The following table represents a reconciliation of the derivative liability recorded in connection with the issuance of the 2019 Notes (in thousands):

January 1, 2019	$	—
Fair value recognized upon issuance of 2019 Notes		247
Fair value adjustment		641
Derivative liability extinguished upon conversion		(438)
December 31, 2019	$	450

During 2018, a derivative liability was initially recorded as a result of the issuance of the 2018 Notes. (see note 12). 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 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 be 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 2018 Notes (in thousands):

January 1, 2018	$	—
Fair value recognized upon issuance of 2018 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, 2019 and 2018, was approximately $35.1 million and $25.2 million, respectively. At December 31, 2019 and 2018, the Company estimates that the fair value of its long-term debt, including the current portion, was approximately $37.0 and $26.9 million, respectively. The fair value of the Company's long-term debt was determined using Level 3 inputs.

The fair value of the 2018 Notes and 2019 Notes was approximately $12.5 million and $50.5 million, respectively, as of December 31, 2019, 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 2018 Notes and 2019 Notes was determined using Level 2 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.

There were no realized gains or losses on investments for the years ended December 31, 2019, 2018 or 2017. There were two debt securities and fourteen debt securities in an unrealized loss position as of December 31, 2019 and December 31, 2018, respectively. None of these investments had been in an unrealized loss position for more than 12 months as of December 31, 2019 or December 31, 2018, respectively. The fair value of these securities as of December 31, 2019 and December 31, 2018 was $5.8 million and $46.9 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, 2019 and December 31, 2018, respectively.

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

	December 31, 2019			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Cash, cash equivalents & restricted cash:				
Cash and money market accounts	$ 77,764	$ —	$ —	$ 77,764
Corporate bonds and commercial paper (due within 90 days)	1,498	—	—	1,498
Total cash and cash equivalents	$ 79,262	$ —	$ —	$ 79,262
Investments:				
Corporate bonds and commercial paper (due within 1 year)	$ 31,979	$ 14	$ —	$ 31,993
Total investments	$ 31,979	$ 14	$ —	$ 31,993
Total cash, cash equivalents and investments	**$ 111,241**	**$ 14**	**$ —**	**$ 111,255**

	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**

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, 2019, the Company's cash, cash equivalents and investments were deposited at three 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, 2019 and 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, 2019 and 2018, four customers and two customers, respectively, 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 impairment losses have been recorded through December 31, 2019.

Other assets

Other assets primarily consist of prepayments made to contract research organizations (CROs). As of December 31, 2019 and 2018, other assets were primarily comprised of approximately $755,000 of prepaid CRO expenses that the Company assumed and paid to 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 recognizes stock-based compensation expense for stock options, and restricted stock units (RSUs) issued to employees and directors based on the grant date fair value of the awards on a straight-line basis over the requisite service period, which typically is the vest period. The Company recognized stock-based compensation for shares issued to employees under our employee stock purchase plan (ESPP) plan Historically, the Company recorded stock-based compensation expense for stock options and RSUs 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. Effective January 1, 2019, the Company recognizes stock-based compensation expense for stock options and RSUs issued to non-employees based on the grant date fair value of the awards on the straight-line basis over the requisite service period. Awards subject to performance-based vesting requirements are expensed utilizing an accelerated attribution model if achievement of the performance criteria is determined to be probable.

The grant date fair value of 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 population. The Company has 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.

The Company issues shares under the Company’s employee stock purchase plan (ESPP) to employees. Stock-based compensation expense for discounted purchases under the ESPP is measured using the Black-Scholes

model to compute the fair value of the lookback provision plus the purchase discount and is recognized as compensation expense over the offering period.

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.

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.

Leases

Effective January 1, 2019, the Company adopted FASB ASC Topic 842, *Leases* (ASC 842). This standard 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.

At the inception of an arrangement, the Company determines whether the arrangement is or contains a lease based on the unique facts and circumstances within the arrangement. A lease is identified where an arrangement conveys the right to control the use of identified property, plant, and equipment for a period of time in exchange for consideration. Leases which are identified within the scope of ASC 842 and which have a term greater than one year are recognized on the Company's consolidated balance sheets as right-of-use assets, lease liabilities and, if applicable, long-term lease liabilities. The Company has elected not to recognize leases with terms of one year or less on its consolidated balance sheets. Operating lease liabilities and their corresponding right-of-use assets are recorded based on the present value of lease payments over the expected remaining lease term. However, certain adjustments to the right-of-use asset may be required for items such as initial direct costs paid or incentives received. The interest rate implicit in lease contracts is typically not readily determinable. As a result, the Company utilizes its incremental borrowing rates to calculate the present value of lease payments. Incremental borrowing rates are the rates the Company incurs to borrow on a collateralized basis over a similar term an amount equal to the lease payments in a similar economic environment.

In accordance with ASC 842, components of a lease are split into three categories: lease components (e.g., land, building, etc.), non-lease components (e.g., common area maintenance, maintenance, consumables, etc.), and non-components (e.g., property taxes, insurance, etc.). The fixed and in-substance fixed contract consideration (including any related to non-components) must be allocated based on fair values to the lease components and non-lease components. Although separation of lease and non-lease components is required, certain practical expedients are available. Entities may elect the practical expedient to not separate lease and non-lease components. Rather, they would account for each lease component and the related non-lease component together as a single component. The Company has elected to account for the lease and non-lease components of each of its operating leases as a single lease component and allocate all of the contract consideration to the lease component only. The lease component results in an operating right-of-use asset being recorded on the consolidated balance sheets and amortized on a straight-line basis as lease expense.

Revenue Recognition

The Company recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the Company expects to receive in exchange for those goods or services, in accordance with ASC 606 *Revenue from Contracts with Customers*. To determine revenue recognition contracts with its customers, the Company 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, 2019, 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.

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, 2019.

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, 2019.

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.

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

As of December 31, 2019, the Company has not received any returns.

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, 2019.

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 (i) licenses, or options to obtain licenses, to the Company's 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 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 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 16, *License and collaboration agreements*.

Accounts Receivable, Net

Accounts receivable, net consists of amounts due from customers, net of applicable revenue reserves. Accounts receivable have standard payments that generally require payment within 30 to 90 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, 2019.

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 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 sales - product. 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 sales - product in the consolidated statements of operations and comprehensive loss.

Shipping and handling costs for product shipments are recorded as incurred in cost of sales - product 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 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 13 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 2016, the FASB issued ASU No. 2016-13, *Measurement of Credit Losses on Financial Instruments* ("ASU 2016-13"). ASU 2016-13 will replace the incurred loss impairment methodology under current GAAP with a methodology that reflects expected credit losses and requires consideration of a broader range of reasonable and supportable information to inform credit loss estimates. In November 2019, the FASB issued ASU *2019-10, Financial Instruments – Credit Losses (Topic 326), Derivatives (Topic 815), and Leases (Topic 842)*. This ASU delayed the required adoption for SEC filers that are smaller reporting companies as of their determination on November 15, 2019, until annual and interim periods beginning after December 15, 2022, with early adoption permitted. The Company has determined that as of November 15, 2019, it is a smaller reporting company and has not elected to early adopt this standard. The Company is currently evaluating the impact the adoption of the standard will have on its consolidated financial statements and related disclosures.

In December 2019, the FASB issued ASU No 2019-12, *Simplifying Accounting for Income Taxes* (ASU 2019-12). ASU 2019-12 removes certain exceptions for recognizing deferred taxes for investments, performing intraperiod allocation, calculating income taxes in interim periods and adds certain guidance to remove complexity in certain areas. ASU 2019-12 is effective for all entities for annual and interim periods beginning after December 15, 2020. 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.

Recently Adopted Accounting Standards Updates

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 was 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 adopted this standard prospectively effective January 1, 2019. The adoption of this ASU did not have an effect on the Company's consolidated financial statements or 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* (ASC 842). 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 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 adopted this standard using the optional transition method effective January 1, 2019. Upon adoption of this standard, the Company recognized a lease liability and a corresponding right-of use asset of $4.0 million and $3.4 million, respectively, and derecognized a deferred rent liability and a corresponding lease incentive obligation of $0.4 million and $0.2 million, respectively. The Company did not record any cumulative effect adjustment to accumulated deficit as a result of adopting this standard. The Company also elected to adopt the practical expedients upon transition, which permit companies to not reassess lease identification, classification, and initial direct costs under ASU 2016-02 for leases that commenced prior to the effective date.

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 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, 2019	December 31, 2018
Raw materials	$ 955	$ —
Work in process	2,040	63
Finished goods	101	264
Total inventories	**$ 3,096**	**$ 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, 2019	December 31, 2018
Leasehold improvements	$ 146	$ 146
Furniture and fixtures	1,074	1,074
Computer equipment	665	658
	1,885	1,878
Less: accumulated depreciation	(938)	(509)
Total property and equipment, net	**$ 947**	**$ 1,369**

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 $0.4 million, $1.0 million, and $0.6 million in depreciation expense for the years ended December 31, 2019, 2018 and 2017, respectively.

5. Intangible assets

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

	December 31, 2019	Estimated useful life
Acquired and in-licensed rights	$ 22,000	14 years
Less: accumulated amortization	(1,992)	
Total intangible assets, net	**$ 20,008**	

Acquired and in-licensed rights as of December 31, 2019, 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 $1.6 million and $0.4 million in amortization expense related to finite-lived intangible assets during the year ended December 31, 2019 and December 31, 2018, respectively, using straight-line methodology. Estimated future amortization expense for finite-lived intangible assets as of December 31, 2019 is approximately $1.6 million per year thereafter.

6. Accrued expenses

Accrued expenses consist of the following (in thousands):

	December 31, 2019	December 31, 2018
Compensation and related benefits	7,399	8,749
Contract research organization costs	5,467	6,682
Commercialization costs	3,028	1,979
Interest	897	1,786
Consulting fees	1,610	494
Professional fees	573	482
Other	391	936
Total accrued expenses	**$ 19,365**	**$ 21,108**

7. Long-term debt

On March 21, 2017 (Closing Date), the Company 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. The Original Loan Agreement was amended on January 4, 2018, March 6, 2018, and October 11, 2018, (the Amended Loan Agreement) to increase the total borrowing limit under the Original Loan Agreement from $25.0 million up to $50.0 million (the Amended Term Loan), pursuant to certain conditions of funding.

On April 23, 2019 (the Fourth Amendment Date) and November 14, 2019 (the Fifth Amendment Date), the Company entered into the Fourth Amendment and Fifth Amendment (together the Amendments) to the Original Loan Agreement with Hercules. The Amendments amend the Amended Loan Agreement (together, with the Amendments, the 2019 Term Loan Agreement).

Per the terms of the 2019 Term Loan Agreement, the Company may borrow up to an aggregate of $75.0 million, of which $35.0 million was outstanding immediately as of the Fourth Amendment Date (2019 Term A Loan) as a result of the existing outstanding principal of term loans of $25.0 million under the Amended Loan Agreement being converted into the 2019 Term A Loan, and an additional $10.0 million being drawn on the Fourth Amendment Date. The remaining $40.0 million of borrowing capacity may be drawn in multiple tranches comprised of (i) a term loan in an amount of up to $15.0 million upon us generating cumulative net product revenues (as defined in the 2019 Term Loan Agreement) of either (a) $37.5 million on or before April 30, 2020 or (b) $50.0 million on or before June 30, 2020 (2019 Term B Loan), and (ii) a term loan in an amount of up to $25.0 million available through December 31, 2021, subject to Hercules' approval and certain other conditions specified in the 2019 Term Loan Agreement (the 2019 Term C Loan, and together with the 2019 Term A Loan and 2019 Term B Loan, the 2019 Term Loan). As of December 31, 2019, The Company has borrowed a total of $35.0 million in term loans.

The Fifth Amendment modified the financial covenants and collateral requirements. As of the Fifth Amendment Date, the Company must maintain cash in an aggregate amount greater than or equal to 100% of the outstanding term loans as collateral, until the Company's receipt of Net Product Revenues (as defined in the 2019 Term Loan Agreement) of at least $20 million on or before December 31, 2020, measured on a trailing six month basis (Initial Net Product Revenue Threshold). As of December 31, 2019 the Company has not met the Initial Net Product Revenue Threshold and has recorded $35.0 million as non-current restricted cash on the consolidated balance sheet.

The 2019 Term Loan will mature on December 1, 2022 (2019 Term Loan Maturity Date). Each advance accrues interest at a floating per annum rate equal to the greater of (a) 9.75% or (b) the lesser of (i) 12.00% and (ii) the sum of (x) 9.75% plus (y) (A) the prime rate minus (B) 5.50%. The 2019 Term Loan provides for interest-only payments until April 1, 2021, which may be extended to December 1, 2021 pursuant to us generating $40.0 million

in net product revenue on a trailing six-month basis on or prior to December 31, 2020 provided that no event of default has occurred. Thereafter, amortization payments will be payable monthly in equal installments of principal and interest (subject to recalculation upon a change in prime rates).

The 2019 Term Loan is secured by a lien on substantially all of our assets, other than intellectual property and contains customary covenants and representations, including a liquidity covenant, minimum net revenue covenant, financial reporting covenant and limitations on dividends, indebtedness, collateral, investments, distributions, transfers, mergers or acquisitions, taxes, corporate changes, deposit accounts, and subsidiaries.

On the Fourth Amendment Date, the Company was required to pay any outstanding accrued interest as well as the final payment fee equal to 4.5% on the outstanding principal balance of the Amended Term Loan, or $1.1 million. No prepayment charges were due as a result of executing the Amendment or conversion of the existing term loans into 2019 Term A Loans.

The events of default under the 2019 Term 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 2019 Term Loan Agreement, promissory notes or other loan documents, (ii) any breach or default in the performance of any covenant under the 2019 Term 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 2019 Term Loan Agreement.

The 2019 Term Loan Agreement also contains other customary provisions, such as expense reimbursement and confidentiality. Hercules has indemnification rights and the right to assign the 2019 Term Loan.

The Company assessed all terms and features of the 2019 Term 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 2019 Term Loan Agreement, including put and call features. The Company determined that all features of the 2019 Term 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, 2019.

The future principal payments under the 2019 Term Loan Agreement are as follows as of December 31, 2019 (in thousands):

2021	$	14,234
2022		20,766
Total principal payments	**$**	**35,000**

8. Product revenue reserves and allowances

As of December 31, 2019, 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, 2019 (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)
Balance at December 31, 2018	$ 29	$ 88	$ 157	$ 2	$ 276
Provision related to sales in the current year	512	1,306	608	74	2,500
Adjustments related to prior period sales	—	—	(76)	—	(76)
Credits and payments made	(430)	(1,139)	(317)	—	(1,886)
Ending balance at December 31, 2019	**$ 111**	**$ 255**	**$ 372**	**$ 76**	**$ 814**

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. Leases

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 Company has accounted for its Needham, Massachusetts office space as an operating lease. The Company's lease contains an option to renew and extend the lease terms and an option to terminate the lease prior to the expiration date. The Company has not included the lease extension or the termination options within the right-of-use asset and lease liability on the consolidated balance sheets as neither option is reasonably certain to be exercised. The Company's lease includes variable non-lease components (e.g., common area maintenance, maintenance, consumables, etc.) that are not included in the right-of-use asset and lease liability and are reflected as an expense in the period incurred. The Company does not have any other operating or finance leases.

In calculating the present value of future lease payments, the Company has elected to utilize its incremental borrowing rate based on the remaining lease term at the date of adoption of ASC 842. The Company has elected to account for lease components and associated non-lease components as a single lease component and has allocated all of the contract consideration to the lease components only. This will potentially result in the initial and subsequent measurement of the balances of the right-of-use asset and lease liability for leases being greater than if the policy election was not applied.

As of December 31, 2019 a right-of-use asset of $3.1 million and lease liability of $3.9 million are reflected on the consolidated balance sheets. The elements of lease expense were as follows (dollar amounts in thousands):

	Year ended December 31, 2019
Lease Expense	
Operating lease expense	$ 885
Total Lease Expense	**$ 885**
Other Information - Operating Leases	
Operating cash flows paid for amounts included in measurement of lease liabilities	$ 703

	December 31, 2019
Other Balance Sheet Information - Operating Leases	
Weighted average remaining lease term (in years)	5.5
Weighted average discount rate	14.60%
Maturity Analysis	
2020	$ 955
2021	1,019
2022	1,039
2023	1,060
2024	1,081
Thereafter	546
Total	**$ 5,700**
Less: Present value discount	(1,791)
Lease Liability	**$ 3,909**

The Company adopted ASU 2016-02 effective January 1, 2019 using the optional transition method permitted under ASU 2018-11. Accordingly, periods presented prior to January 1, 2019 were not restated to reflect the accounting principles adopted under ASU 2016-02. Prior to adoption, the Company recorded rent expense from its Needham office on a straight-line basis over the term of the lease with the deferred rent obligation included in accrued expenses (current portion) and other liabilities (noncurrent portion) in the consolidated balance sheet as of December 31, 2018. The Company amortized any leasehold improvements over the lesser of the useful life of those improvements or the life of the lease. For the year ended December 31, 2018, the Company recorded rent expense of $0.8 million.

At December 31, 2018, future minimum lease payments under non-cancelable leases under ASC 840 were as follows (in thousands):

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

10. Common stock

As of December 31, 2019 and 2018, 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,	
	2019	2018
Shares reserved under equity compensation plans	15,389	15,572
Shares reserved for inducement grants	6,331	6,381
Shares reserved for 2018 Notes	3,950	20,937
Shares reserved for 2019 Notes	34,796	—
Total shares reserved	60,466	42,890

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 (ATM) 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. 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 ATM to $75.0 million. Through December 31, 2018, the Company sold 11,518,354 shares under the ATM for net proceeds of approximately $47.3 million (after deducting commissions and other offering expenses). During the year ended December 31, 2019, there were no sales under the ATM. As of December 31, 2019 we can issue an additional $26.6 million of gross proceeds under this program.

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.

11. 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,		
	2019	2018	2017
Research and development	$ 1,501	$ 2,043	$ 1,381
Selling, general and administrative	7,038	4,628	3,652
Total stock-based compensation expense	**$ 8,539**	**$ 6,671**	**$ 5,033**

All of the $8.5 million, $6.7 million, and $5.0 million of stock-based compensation expense recorded during the years ended December 31, 2019, 2018 and 2017, respectively, was recorded to additional paid-in capital.

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 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, 2019, under the 2012 Plan, the Company has granted stock options for 19,733,446 shares of common stock, of which 5,644,811 have been forfeited and 456,865 have been exercised, and granted restricted stock units for 1,922,622 shares of common stock, of which 443,277 have been forfeited and 851,256 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, 2019, the Company had granted options for 6,094,671 shares of common stock under the program, of which 2,212,738 have been forfeited and 323,750 have been exercised, and granted restricted stock units for 162,200 shares, of which 62,200 have been forfeited and 50,000 have vested. As of December 31, 2019, 3,608,183 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, 2019 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, 2018	12,522,867	$ 5.42	7.8	$ 6,909
Granted	8,280,682	$ 2.09		
Exercised	(91,907)	$ 1.41		
Forfeited/cancelled	(3,453,118)	$ 4.67		
Outstanding at December 31, 2019	17,258,524	$ 4.00	7.3	$ 185
Vested at December 31, 2019	7,641,065	$ 5.45	5.3	$ 121
Vested and expected to vest at December 31, 2019(1)	16,728,024	$ 4.00	7.3	$ 185

(1) This represents the number of vested options as of December 31, 2019, plus the number of unvested options expected to vest as of December 31, 2019.

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

	Year ended December 31,		
	2019	2018	2017
Risk-free interest rate	1.98 %	2.65 %	2.02 %
Volatility	87 %	81 %	78 %
Dividend yield	—	—	—
Expected term (years)	5.9	5.8	5.8

The Company recorded stock-based compensation expense associated with employee stock options of $7.1 million, $5.6 million, and $4.5 million for the years ended December 31, 2019, 2018, and 2017, respectively. The weighted-average grant date fair value of options granted in the years ended December 31, 2019, 2018, and 2017 was $1.44, $3.72, and $1.83 per share, respectively. The fair value of options that vested during the years ended December 31, 2019, 2018, and 2017 was $7.3 million, $4.7 million, and $4.8 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, 2019 and 2018 was $0.1 million and $1.8 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 $0.1 million and $0.7 million of stock-based compensation expense during the year ended December 31, 2019 and 2018, respectively, related to awards that vest upon the achievement of performance conditions. As December 31, 2019, a total of 260,000 performance-based options remain unvested which are expected to vest, which have a weighted average exercise price of $1.52 per share, weighted average remaining contractual term of 9.6 years and $0 aggregate intrinsic value.

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, 2019, there was $14.0 million of total unrecognized compensation cost related to unvested stock options and the Company expects to recognize this cost over a remaining weighted-average period of 2.8 years.

Restricted Stock Units (RSUs)

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.

A summary of RSU activity during the year ended December 31, 2019 is as follows:

	Shares	Weighted-average grant date fair value per share
Outstanding at December 31, 2018	306,750	$ 5.24
Granted	798,904	$ 2.57
Vested	(141,439)	$ 6.93
Forfeited/cancelled	(286,126)	$ 3.86
Outstanding at December 31, 2019	678,089	$ 2.36

The Company recorded stock-based compensation expense associated with employee RSUs of $1.0 million, $0.4 million, and less than $0.1 million for the years ended December 31, 2019, 2018, and 2017, respectively. No RSUs were granted during the years ended December 31, 2017. The total fair value of restricted stock units vested during the years ended December 31, 2019, 2018, and 2017 was approximately $0.3 million, $0.0 million, and $0.0 million, respectively.

At December 31, 2019, 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 2.6 years.

Employee stock purchase plan

At the Special Meeting of Stockholders, held on December 18, 2018, the stockholders approved the 2018 Employee Stock Purchase Plan (2018 ESPP). On June 21, 2019, the board of directors of the Company amended and restated the 2018 ESPP, to account for certain non-material changes to the plan's administration (the Amended and Restated 2018 ESPP). The Amended and Restated 2018 ESPP provides eligible employees with the opportunity, through regular payroll deductions, to purchase shares of the Company's common stock at 85% of the lesser of the fair market value of the common stock (a) on the date the option is granted, which is the first day of the purchase period, and (b) on the exercise date, which is the last business day of the purchase period. The Amended and Restated 2018 ESPP generally allows for two six-month purchase periods per year beginning in January and July, or such other periods as determined by the compensation committee of our board of directors. The Company has

reserved 2,000,000 shares of common stock for the administration of the Amended and Restated 2018 ESPP. The fair value of shares expected to be purchased under the Amended and Restated 2018 ESPP was calculated using the Black-Scholes model with the following assumptions:

	Six Months ended June 30, 2019	Six Months ended December 31, 2019
Risk-free interest rate	2.46 %	2.10 %
Volatility	79 %	95 %
Dividend yield	—	—
Expected term (years)	0.4	0.5

For the year ended December 31, 2019, the Company has recognized $0.4 million of stock-based compensation expense under the Amended and Restated 2018 ESPP. During the year ended December 31, 2019, the Company issued 341,701 shares of common stock for proceeds of $0.4 million under the Amended and Restated 2018 ESPP.

12. 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 2018 Notes), for net proceeds of approximately $145.3 million. The 2018 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 2018 Base Indenture, the 2018 Indenture), each dated October 17, 2018, by and between the Company and Wilmington Trust, National Association, as trustee. The 2018 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 2018 Notes will mature on November 1, 2048, unless earlier repurchased, redeemed or converted in accordance with their terms.

The 2018 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 2018 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 2018 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 2018 Indenture), whether or not consecutive, during any 30 consecutive VWAP Trading Day period commencing on or after the date the Company first issued the 2018 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 2018 Notes. On or after November 1, 2022, the Company may elect to redeem the 2018 Notes, in whole or in part, at a cash redemption price equal to the principal amount of the 2018 Notes to be redeemed, plus accrued and unpaid interest, if any.

Unless the Company has previously called all outstanding 2018 Notes for redemption, the 2018 Notes will be subject to repurchase by the Company at the holders' option on each of November 1, 2023, November 1, 2028, 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 2018 Notes to be repurchased, plus accrued and unpaid interest, if any.

If a “Fundamental Change” (as defined in the 2018 Indenture) occurs at any time, subject to certain conditions, holders may require the Company to purchase all or any portion of their 2018 Notes at a purchase price equal to 100% of the principal amount of the 2018 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 2018 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 2018 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 2018 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 2018 Indenture includes customary covenants and set forth certain events of default after which the 2018 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 2018 Notes become automatically due and payable

The Company assessed all terms and features of the 2018 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 2018 Notes, including the conversion, put and call features. Per the terms of the 2018 Indenture, upon conversion of the 2018 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 2018 Notes with the conversion option and the fair value of the 2018 Notes without the conversion option. The Company determined that the fair value of the derivative upon issuance of the 2018 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 2018 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 2018 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.

On November 14, 2019 and December 23, 2019, we entered into privately negotiated agreements to exchange approximately $114.3 million and $7.4 million, respectively, aggregate principal amount of the 2018 Notes for (i) approximately $62.9 million and $4.0 million, respectively, aggregate principal amount of 5.00% Convertible Senior Second Lien Notes due 2048 (the 2019 Notes) (ii) an aggregate of approximately $11.4 million and $0.7 million in 2018 Notes principal repayment and (iii) accrued interest on the 2018 Notes through November 14, 2019 and December 23, 2019, respectively. The 2019 Notes are governed by the terms of an indenture (the 2019 Indenture). The 2019 Notes are senior secured obligations of the Company and bear interest at 5.00% per annum,

payable semi-annually in arrears on May 1 and November 1 of each year. The 2019 Notes will mature on November 1, 2048, unless earlier repurchased, redeemed or converted in accordance with the terms.

The Company determined 2019 Notes exchange met the definition of a troubled debt restructuring under ASC 470-60, *Troubled Debt Restructurings by Debtors*, as the Company was experiencing financial difficulties and the lenders granted a concession. The future undiscounted cash flows of the 2019 Notes after the exchange exceeded the carrying value of the converted 2018 Notes prior to the exchange. As such no gain was recognized as a result of the exchange. The Company reduced the carrying value of the Notes by the cash given and the change in fair value of the conversion option driven by the reduction in conversion price. The change in fair value of the conversion option was determined to be $13.6 million.

The 2019 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 606.0606 shares of common stock per $1,000 principal amount of the 2019 Notes, which corresponds to an initial conversion price of approximately $1.65 per share of common stock and represents a conversion premium of approximately 52.8% above the last reported sale price of our common stock of $1.08 per share on November 11, 2019.

We will have the right, exercisable at our option, to cause all 2019 Notes then outstanding to be converted automatically if the “Daily VWAP” (as defined in the 2019 Indenture) per share of our common stock equals or exceeds 121% 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 2019 Notes. (Company’s Mandatory Conversion Option)

Upon conversion, converting noteholders will be entitled to receive accrued interest on their converted 2019 Notes. In addition, if the 2019 Notes are converted with a conversion date that is on or prior to November 1, 2020, other than in connection with the Company’s exercise of the Company’s Mandatory Conversion Option then the consideration due upon any such conversion will also include a cash interest make-whole payment for all future scheduled interest payments on the converted 2019 Notes through November 1, 2020 (2019 Notes Interest Make-Whole Provision).

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 2019 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 2019 Notes, including the conversion, put and call features. In consideration of the 2019 Notes Interest Make-Whole Provision, we concluded the provision required bifurcation as a derivative. The fair value of the 2019 Interest Make-Whole Provision was determined using a Monte Carlo model. It was determined that the fair value of the derivative upon the November 14, 2019 and December 23, 2019 issuance of the 2019 Notes was $0.2 million in aggregate; 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 2019 Notes on the closing dates. During the period November 14, 2019 to December 31, 2019, the Company paid out approximately $0.4 million in 2019 Interest Make-Whole payments which was recorded as a reduction of the derivative liability. As of December 31, 2019, we determined the fair value of the 2019 Interest Make-Whole Provision was $0.5 million. The Company recorded the change in the fair value of the 2019 Interest Make-Whole Provision for the period from November 14, 2019 to December 31, 2019 of $0.6 million as other expense on the consolidated statements of operations and comprehensive loss.

The Company determined that all other features of the 2018 Notes and 2019 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, 2019.

The Company determined that the expected life of the 2018 Notes and 2019 Notes was equal to the period through November 1, 2023 as this represents the point at which the 2018 Notes and 2019 Notes are initially subject to repurchase by the Company at the option of the holders. Accordingly, the total debt discount, inclusive of the fair value of the embedded conversion feature derivative at issuance, is being amortized using the effective interest method through November 1, 2023. For the year ended December 31, 2019, the Company recognized an aggregate of $16.0 million of interest expense related to the 2018 and 2019 Notes. For the year ended December 31, 2019, 2019 Note holders converted $9.5 million aggregate principal of 2019 Notes in exchange for 5,767,872 shares of common stock and $0.4 million of cash for 2019 Interest Make-Whole Provision.

13. 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 years ended December 31, 2019 and 2017 net loss, basic and diluted EPS are the same as the assumed exercise of stock options, restricted stock units, and the Notes are anti-dilutive. 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.

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

	Year Ended December 31,		
	2019	2018	2017
Net loss	(149,209)	(72,434)	(67,802)
Less: Other income	—	(25,556)	—
Add: Interest expense	—	3,071	—
Adjusted diluted net loss	**(149,209)**	**(94,919)**	**(67,802)**
Weighted average shares outstanding	74,578	64,962	38,422
Add: Dilutive effect of the Notes	—	4,359	—
Weighted average diluted shares outstanding	**74,578**	**69,321**	**38,422**
Net loss per share - basic	(2.00)	(1.12)	(1.76)
Net loss per share - diluted	(2.00)	(1.37)	(1.76)

For the year ended December 31, 2018, 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,		
	2019	2018	2017
Outstanding stock options	17,258,524	12,522,867	8,719,978
Outstanding restricted stock units	678,089	306,750	—
2018 Notes	3,950,032	—	—
2019 Notes	34,796,363	—	—
Total potentially dilutive securities	**56,683,008**	**12,829,617**	**8,719,978**

14. Income Taxes

As of December 31, 2019, the Company had federal and state net operating loss carryforwards of approximately $371.7 million and $392.3 million, respectively, which are available to reduce future taxable income. The Company also had federal and state tax credits of $20.6 million and $3.0 million, respectively, which may be used to offset future tax liabilities. The net operating loss (NOL) and tax credit carryforwards will expire at various dates through 2039, except for $136.3 million of federal net operating loss carryforwards which may be carried forward indefinitely. 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,	
	2019	2018
Income tax benefit using U.S. federal statutory rate	21.00 %	21.00 %
State tax benefit, net of federal benefit	4.26 %	6.38 %
Research and development tax credits	1.67 %	5.61 %
Cancellation of debt	(4.76)%	— %
Permanent items	(1.41)%	(0.65)%
Change in the valuation allowance	(20.33)%	(31.82)%
Other	(0.43)%	(0.52)%
	— %	— %

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

	December 31, 2019	December 31, 2018
Deferred tax assets:		
Net operating loss carryforwards	$ 101,039	$ 88,829
Capitalized research and development	2,230	2,545
Research and development credits	22,944	19,725
Stock-based compensation	4,445	3,756
Other	1,451	543
Total deferred tax assets	132,109	115,398
Deferred tax liabilities:		
Debt discount	(3,702)	(13,617)
Total deferred tax liabilities	(3,702)	(13,617)
Net deferred tax asset prior to valuation allowance	128,407	101,781
Valuation allowance	(128,407)	(101,781)
Net deferred tax asset	$ —	$ —

The Company has recorded a valuation allowance against its deferred tax assets at December 31, 2019 and 2018 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 $26.6 million in the year ended December 31, 2019 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, 2019, the Company had no unrecognized tax benefits or related interest and penalties accrued. The Company has not conducted a study of research and development (R&D) credit carryforwards. This study may result in an adjustment to the Company's R&D credit carryforwards; however, until a study is completed and any adjustment is known, no amounts are being presented as an uncertain tax position. A full valuation allowance has been provided against the Company's R&D credits and, if an adjustment is required, this adjustment would be offset by an adjustment to the valuation allowance. Thus, there would be no impact to the consolidated balance sheet or statement of operations if an adjustment were required. The Company would recognize both accrued interest and penalties related to unrecognized benefits in income tax expense. The Company's uncertain tax positions are related to years that remain subject to examination by relevant tax authorities. Since the Company is in a loss carryforward position, the Company is generally subject to examination by the U.S. federal, state and local income tax authorities for all tax years in which a loss carryforward is available.

15. Commitments and contingencies

The Company has entered into a lease agreement for approximately 27,810 square feet of office space in Needham, Massachusetts. Please refer to Note 9 for further details regarding the minimum aggregate future lease commitments as of December 31, 2019. In conjunction with the execution of the Amended Lease Agreement, the Company has provided a security deposit in the form of a letter of credit in the amount of $0.2 million as of December 31, 2019 and December 31, 2018. The amount is included in non-current restricted cash on the consolidated balance sheets as of December 31, 2019.

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.

16. License and collaboration agreements

Infinity Pharmaceuticals, Inc. (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 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 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 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.

On March 5, 2019, Infinity and Healthcare Royalty Partners III, L.P. (HCR) entered into a purchase and sale agreement, in which HCR paid Infinity a $30.0 million upfront payment and is entitled to receive up to $20.0 million in potential milestone payments from Infinity. In exchange HCR has received the right to receive the royalties due to Infinity from us under the license agreement. As a result, we now pay royalties previously due to Infinity to HCR. We will continue to pay Infinity for the royalties due to MICL and Purdue described above.

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, 2019 and 2018, the Company recorded royalty expense of $1.0 million, and $0.1 million, respectively related to the HCR, Infinity, MICL, and Purdue royalty payments, which are included in costs of sales - product within the consolidated statements of operation. There were no royalties paid to Infinity or HCR during the year ended December 31, 2017.

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, 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.

Subsequently, on February 28, 2019, the Company entered into a supply agreement with Yakult (the Yakult Supply Agreement), under which the Company agreed to provide Yakult with drug product for clinical and commercial use in accordance with the Yakult Agreement. Under the terms of the Yakult Supply Agreement, the Company also granted to Yakult a limited manufacturing license to fill, finish, package, and label the drug product solely for clinical and commercial purposes in Japan.

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 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 has recognized $0.1 million of collaboration revenue under the Yakult Supply Agreement for the year ended December 31, 2019. 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 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 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.

For the year ended December 31, 2019 there have been no additional milestones achieved under the CSPC Agreement. 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.

Sanofi

On July 25, 2019, the Company entered into a license and collaboration agreement with Sanofi (the Sanofi Agreement), under which the Company granted exclusive rights to Sanofi to develop and commercialize products containing duvelisib in Russia, the Commonwealth of Independent States (CIS), Turkey, the Middle East and Africa (collectively the "Sanofi Territory") for the treatment, prevention, palliation or diagnosis of any oncology indication in humans or animals.

Under the terms of the Sanofi Agreement, Sanofi received the exclusive right to develop and commercialize products containing duvelisib in the Sanofi Territory under mutually agreed upon development and commercialization plans at Sanofi's own cost and expense. In addition, Sanofi received certain limited manufacturing rights in the event the Company is unable to manufacture or supply sufficient quantities of duvelisib or products containing duvelisib to Sanofi during the term of the Sanofi Agreement. The Company retained all rights to duvelisib outside the Sanofi Territory, except for those territories previously and exclusively licensed to other partners.

Sanofi paid the Company an upfront, non-refundable payment of $5.0 million in August 2019. The Company is also entitled to receive aggregate payments of up to $42.0 million if certain regulatory and commercial milestones are successfully achieved. Sanofi is obligated to pay the Company double-digit royalties on net sales of products containing duvelisib in the Sanofi Territory, subject to reduction in certain circumstances.

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

The Company first assessed the Sanofi Agreement under ASC 808 to determine whether the Sanofi Agreement (or part of the Sanofi Agreement) represents a collaborative arrangement based on the respective risks, rewards and activities of the parties. 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. The Company concluded that the Sanofi Agreement (or part of the Sanofi Agreement) does not represent a collaborative arrangement under ASC 808. The Company then considered each component in the Sanofi Agreement to determine if ASC 606 should be applied to those components. Generally, the component in the Sanofi Agreement that falls under potential research and development activities is the development of duvelisib specifically in the Sanofi Territory.

For development of duvelisib specifically in the Sanofi Territory, the Company has concluded that Sanofi is a customer with regard to this component in the context of the Sanofi Agreement. As such, the Sanofi Territory component and all related payments are within the scope of ASC 606.

The Company determined that there were two material promises associated with the Sanofi territory-specific activities: (i) an exclusive license to develop and commercialize duvelisib in the Sanofi Territory and (ii) the initial technology transfer. The Company determined that the exclusive license and initial technology transfer were not distinct from one another, as the license has limited value without the initial technology transfer. 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 Sanofi 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 that one performance obligation exists at the outset of the Sanofi Agreement, which is the exclusive license combined with the initial technology transfer.

The Company has determined that the upfront payment of $5.0 million constituted the transaction price at the outset of the Sanofi 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 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 $5.0 million as license and collaboration revenue during the year ended December 31, 2019.

17. 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 $1.3, $0.8 million, and $0.3 million for the years ended December 31, 2019, 2018, and 2017, respectively.

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

	First Quarter Ended March 31, 2019	Second Quarter Ended June 30, 2019	Third Quarter Ended September 30, 2019	Fourth Quarter Ended December 31, 2019
Revenue:				
Product revenue, net	$ 1,671	$ 3,019	$ 4,032	$ 3,617
License and collaboration revenue	—	117	5,000	—
Total revenue	1,671	3,136	9,032	3,617
Operating expenses:				
Cost of sales - product	$ 158	$ 377	$ 371	$ 332
Cost of sales - intangible amortization	392	392	392	393
Research and development	9,758	11,346	12,219	12,455
Selling, general and administrative	26,033	29,298	22,153	23,728
Total operating expenses	36,341	41,413	35,135	36,908
Loss from operations	(34,670)	(38,277)	(26,103)	(33,291)
Other income/(expense)	—	—	—	(641)
Interest income	1,497	1,268	1,005	611
Interest expense	(4,929)	(5,185)	(5,041)	(5,453)
Net loss	$ (38,102)	$ (42,194)	$ (30,139)	$ (38,774)
Net loss per share —basic	$ (0.52)	$ (0.57)	$ (0.41)	$ (0.51)
Net loss per share —diluted	$ (0.52)	$ (0.57)	$ (0.41)	$ (0.51)
Weighted-average number of common shares used in net loss per share —basic and diluted				
Net loss per share —basic	73,854	73,877	74,228	76,331
Net loss per share —diluted	73,854	73,877	74,228	76,331

	First Quarter Ended March 31, 2018	Second Quarter Ended June 30, 2018	Third Quarter Ended September 30, 2018	Fourth Quarter Ended December 31, 2018
Revenue:				
Product revenue, net	$ —	$ —	$ 508	$ 1,210
License and collaboration revenue	—	10,000	15,000	—
Total revenue	—	10,000	15,508	1,210
Operating expenses:				
Cost of sales - product	$ —	$ —	$ 49	$ 116
Cost of sales - intangible amortization	—	—	31	392
Research and development	10,934	12,381	11,571	8,762
Selling, general and administrative	9,827	15,813	25,426	26,199
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)

(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.

19. 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 sheet date. The Company is not aware of any material subsequent events other than the following:

Chugai Pharmaceutical Co., Ltd. (Chugai) Agreement

On January 7, 2020, the Company entered into a license agreement (the Chugai Agreement) with Chugai Pharmaceutical Co., Ltd. (Chugai) whereby Chugai granted the Company an exclusive worldwide license for the development, commercialization and manufacture of products containing CH5126766, a dual RAF/MEK inhibitor.

Under the terms of the Chugai Agreement, the Company received an exclusive right to develop and commercialize products containing CH5126766 at the Company's cost and expense. Upon execution of the Chugai Agreement, the Company is required to pay Chugai a non-refundable payment of $3.0 million which was paid in February 2020. The Company is further obligated to pay Chugai double-digit royalties on net sales of products containing CH5126766, subject to reduction in certain circumstances. Chugai also obtained opt back rights to develop and commercialize CH5126766 (a) in the European Union, which option may be exercised through the date the Company submits a NDA to the FDA for a product which contains CH5126766 as the sole active pharmaceutical ingredient and (b) in Japan and Taiwan, which option may be exercised through the date the Company receives marketing authorization from the FDA for a product which contains CH5126766 as the sole active pharmaceutical ingredient. As consideration for executing either option, Chugai would have to make a payment to the Company calculated on the Company's development costs to date.

Restructuring

On February 27, 2020, the Company committed to an operational plan to reduce overall operating expenses, including the elimination of approximately 31 positions across the Company and other cost-saving measures (the "Restructuring"). The Restructuring is designed to streamline operations, speed execution of the Company's clinical development of defactinib and CH5126766, and reflect a focused, account-based approach in the field. The Company expects to substantially complete the workforce reduction by the end of the first quarter of 2020.

The Company expects the Restructuring to reduce annualized operating expenses to a range of approximately $70 million to $85 million beginning in the first quarter of 2020.

The Company expects to record a charge of approximately $1.9 million in the first quarter of 2020 as a result of the Restructuring, consisting of one-time termination benefits for employee severance, benefits, and related

costs, all of which are expected to result in cash expenditures and substantially all of which will be paid out over the next three months.

Issuance of Common Stock

On March 3, 2020, the Company sold 46,511,628 shares of Common Stock at a purchase price of $2.15 per share for aggregate gross proceeds of approximately $100.0 million, before deducting fees to the placement agents and other offering expenses payable by the Company pursuant to a securities purchase agreement.

2019 Notes Conversion

From January 1, 2020 through the date of issuance of these consolidated financial statements, the aggregate principal amount of $51.2 million of the Company's 2019 Notes have been converted into 31,044,835 shares of common stock. As a result, as of the date the issuance of these consolidated financials the Company has $6.2 million aggregate principal amount outstanding of 2019 Notes. On March 9, 2020, the Company exercised the Company's Mandatory Conversion Option for the remaining $6.2 million of 2019 Notes outstanding which will require the remaining 2019 Notes to be converted into approximately 3.8 million shares of common stock in March 2020.

DESCRIPTION OF THE REGISTRANT'S SECURITIES REGISTERED PURSANT TO SECTION 12 OF THE SECURITIES EXCHANGE ACT OF 1934

DESCRIPTION OF CAPITAL STOCK

General

The following is a summary of information concerning the capital stock of Verastem, Inc. ("Verastem" or "the Company") that has been registered under the Securities Exchange Act of 1934. The summaries and descriptions below do not purport to be complete and are subject to and qualified in their entirety by reference to the Delaware General Corporation Law, the Company's Amended and Certificate of Incorporation (the "Certificate of Incorporation") and Amended and Restated Bylaws (the "Bylaws") each of which are incorporated by reference as an exhibit to the Annual Report on Form 10-K of which this exhibit is a part. **Common Stock**

Under the Certificate of Incorporation, Verastem has authority to issue up to 200,000,000 shares of common stock, par value $0.0001 per share. As of February 29, 2020, 91,402,823 shares of common stock were issued and outstanding.

Holders of common stock are entitled to one vote for each share held on all matters submitted to a vote of stockholders and do not have cumulative voting rights. An election of directors by the Company's stockholders shall be determined by a plurality of the votes cast by the stockholders entitled to vote on the election. Holders of common stock are entitled to receive proportionately any dividends as may be declared by the Company's board of directors, subject to any preferential dividend rights of outstanding preferred stock.

In the event of the Company's liquidation or dissolution, the holders of common stock are entitled to receive proportionately all assets available for distribution to stockholders after the payment of all debts and other liabilities and subject to the prior rights of any outstanding preferred stock. Holders of common stock have no preemptive, subscription, redemption or conversion rights. The rights, preferences and privileges of holders of common stock are subject to and may be adversely affected by the rights of the holders of shares of any series of preferred stock that the Company may designate and issue in the future.

Delaware Anti-Takeover Law and Certain Charter and Bylaw Provisions

Delaware law

Verastem is subject to Section 203 of the Delaware General Corporation Law. Subject to certain exceptions, Section 203 prevents a publicly-traded Delaware corporation from engaging in a "business combination" with any "interested stockholder" for three years following the date that the person became an interested stockholder, unless either the interested stockholder attained such status with the approval of a corporation's board of directors, the business combination is approved by a corporation's board of directors and stockholders in a prescribed manner or the interested stockholder acquired at least 85% of the outstanding voting stock of the corporation in the transaction in which it became an interested stockholder. A "business combination" includes, among other things, a merger or consolidation involving the Company and an "interested stockholder" and the sale of more than 10% of the Company's assets. In general, an "interested stockholder" is any entity or person beneficially owning 15% or more of the Company's outstanding voting stock and any entity or person affiliated with or controlling or controlled by such entity or person.

Staggered board

Verastem's certificate of incorporation and bylaws divide its board of directors into three classes with staggered three-year terms. In addition, the certificate of incorporation and bylaws provide that directors may be removed only for cause and only by the affirmative vote of the holders of 75% of the shares of capital stock present in person or by proxy and entitled to vote. Under the certificate of incorporation and bylaws, any vacancy on the Company's board

of directors, including a vacancy resulting from an enlargement of the board of directors, may be filled only by vote of a majority of the directors then in office. Furthermore, the certificate of incorporation provides that the authorized number of directors may be changed only by the resolution of the board of directors. The classification of the board of directors and the limitations on the ability of the Company's stockholders to remove directors, change the authorized number of directors and fill vacancies could make it more difficult for a third party to acquire, or discourage a third party from seeking to acquire, control of the Company.

Stockholder action; special meeting of stockholders; advance notice requirements for stockholder proposals and director nominations

Verastem's certificate of incorporation and bylaws provide that any action required or permitted to be taken by its stockholders at an annual meeting or special meeting of stockholders may only be taken if it is properly brought before such meeting and may not be taken by written action in lieu of a meeting. The certificate of incorporation and bylaws also provide that, except as otherwise required by law, special meetings of the stockholders can be called only by the Company's chairman of the board, president or chief executive officer or the board of directors. In addition, the Company's bylaws establish an advance notice procedure for stockholder proposals to be brought before an annual meeting of stockholders, including proposed nominations of candidates for election to the board of directors. Stockholders at an annual meeting may consider only proposals or nominations specified in the notice of meeting or brought before the meeting by or at the direction of the board of directors, or by a stockholder of record on the record date for the meeting who is entitled to vote at the meeting and who has delivered timely written notice in proper form to the Company's secretary of the stockholder's intention to bring such business before the meeting. These provisions could have the effect of delaying until the next stockholder meeting stockholder actions that are favored by the holders of a majority of the Company's outstanding voting securities. These provisions also could discourage a third party from making a tender offer for the Company's common stock, because even if it acquired a majority of the outstanding voting stock, it would be able to take action as a stockholder, such as electing new directors or approving a merger, only at a duly called stockholders meeting and not by written consent.

Super-majority voting

The Delaware General Corporation Law provides generally that the affirmative vote of a majority of the shares entitled to vote on any matter is required to amend a corporation's certificate of incorporation or bylaws, unless a corporation's certificate of incorporation or bylaws, as the case may be, requires a greater percentage. Verastem's bylaws may be amended or repealed by a majority vote of the Company's board of directors or the affirmative vote of the holders of at least 75% of the votes that the Company's stockholders would be entitled to cast in any annual election of directors. In addition, the affirmative vote of the holders of at least 75% of the votes that the Company's stockholders would be entitled to cast in any election of directors is required to amend or repeal or to adopt any provisions inconsistent with any of the provisions of Verastem's certificate of incorporation described above.

Transfer Agent and Registrar

The transfer agent and registrar for the common stock is Computershare Trust Company, N.A.

Listing

The Company's common stock is listed on The Nasdaq Global Market under the symbol "VSTM."

CONSULTANT:	■ Robert Forrester
VERASTEM, INC. CONTACT:	■ Dan Paterson
EFFECTIVE DATE:	■ June 21, 2019

CONSULTING AGREEMENT

This Consulting Agreement (together with its attachments, this "Agreement") made as of the date written above (the "Effective Date") is between **Verastem, Inc**. a Delaware corporation having an address at 117 Kendrick Street, Suite 500, Needham, MA 02494 (the "Company"), and Robert Forrester, residing at 346 Gay Street, Westwood, MA 02142, ("Consultant"). The Company desires to have the benefit of Consultant's knowledge and experience, and Consultant desires to provide Consulting Services (defined below) to the Company, all as provided in this Agreement. All capitalized terms not herein defined shall have the definitions ascribed to them in the Separation Agreement signed between Consultant and the Company on June 25, 2019 (the "Separation Agreement").

1. Consulting Services. The Company hereby retains Consultant in an advisory capacity to be available for advice to the Company from time to time as may be mutually agreed upon and reasonable in light of any other professional obligations the Consultant may undertake. In providing services under this Agreement, Consultant agrees not to use or disclose any trade secrets or other confidential information of any other person, firm, corporation, institution or other entity in connection with any of the Consulting Services without such third party's express written consent. Consultant will comply with all rules, procedures and standards promulgated from time to time by the Company with regard to Consultant's access to and use of the Company's property, information, equipment and facilities. Except as provided in Section 7.2 below, this is a non-cancellable term consulting agreement.

2. Term and Compensation. The term of this Consulting Agreement shall be thirty-seven (37) months, from June 22, 2019 through July 21, 2022. As compensation for the Consulting Services rendered during the Term, the Company will pay Consultant at an hourly rate of $600.00. In further consideration for the Consulting Services rendered by Consultant to the Company, the Company agrees the time within which Consultant shall be allowed to exercise all stock options granted to him pursuant to the terms of the Award Agreements shall be extended until the earlier of July 21, 2022, or the original expiration date of such stock option. Payments will be made by the Company within thirty (30) days from the Company's receipt of Consultant's invoice. Invoices will contain such detail as the Company may reasonably require, and will be payable in U.S. Dollars in accordance with the terms and provisions of the Business Terms. The Company will reimburse Consultant for reasonable and pre-approved business expenses incurred by Consultant in the performance of the Consulting Services as specified in the Business Terms.

3. Inventions.

3.1 Definition. "Inventions" means all inventions, discoveries, improvements, ideas, designs, processes, products, computer programs, works of authorship, databases, gene sequences, cell lines, samples, chemical compounds, assays, biological materials, mask works, trade

secrets, know-how, research and creations (whether or not patentable or subject to copyright or trade secret protection) that Consultant makes, conceives or reduces to practice, either alone or jointly with others, and that (a) result from the performance of the Consulting Services, and/or (b) result from use of facilities, equipment, supplies, Research Materials (defined below), or Confidential Information (defined below) of the Company.

3.2 **Ownership**. Consultant will promptly disclose all Inventions in confidence to the Company. Consultant agrees to irrevocably transfer and assign and hereby does irrevocably transfer and assign to the Company or its successors or designees the entire right, title and interest now existing or that may exist in the future in and to all right, title and interest in and to all Inventions and any and all related patents, patent applications, copyrights, copyright applications, trademarks, trade names, trade secrets and other proprietary and moral rights in the United States and throughout the world developed as a result of and solely as a result of this consulting agreement ("Work Product"). All Work Product will be the exclusive property of the Company. For purposes of the copyright laws of the United States, all Work Product will constitute "works made for hire", except to the extent such Inventions cannot by law be "works made for hire". Consultant agrees to execute, at the Company's request and expense, all documents and other instruments necessary or desirable to confirm such assignment. In the event that Consultant does not, for any reason, execute such documents within a reasonable time of the Company's request, Consultant hereby irrevocably appoints the Company as Consultant's attorney-in-fact for the purpose of executing such documents on Consultant's behalf, which appointment is coupled with an interest. Consultant shall not attempt to register any works created by Consultant pursuant to this Agreement at the U.S. Copyright Office, the U.S. Patent & Trademark Office, or any foreign copyright, patent, or trademark registry. Consultant retains no rights in the Work Product and agrees not to challenge the Company's ownership of the rights embodied in the Work Product. Consultant further agrees to assist the Company in every proper way to enforce the Company's rights relating to the Work Product in any and all countries, including, but not limited to, executing, verifying and delivering such documents and performing such other acts (including appearing as a witness) as the Company may reasonably request for use in obtaining, perfecting, evidencing, sustaining and enforcing the Company's rights relating to the Work Product.

3.3 **Moral Rights.** If Consultant has any rights, including without limitation "artist's rights" or "moral rights" in the Work Product which cannot be assigned (the "Non-Assignable Rights"), Consultant agrees to waive enforcement worldwide of such rights against the Company. In the event that Consultant has any such rights that cannot be assigned or waived, Consultant hereby grants to the Company a royalty-free, paid-up, exclusive, worldwide, irrevocable, perpetual license under the Non-Assignable Rights to (i) use, make, sell, offer to sell, have made, commercialize, and further sublicense the Work Product, and (ii) reproduce, distribute, create derivative works of, publicly perform and publicly display the Work Product in any medium or format, whether now known or later developed.

3.4 **Research Materials**. For Consulting Services which involve laboratory work or experiments, "Research Materials" means all materials (a) furnished by the Company, (b) developed by Consultant in connection with the Consulting Services, or (c) the cost of which are reimbursed to Consultant by the Company. Research Materials include, in the case of biological materials, all progeny and unmodified derivatives of those materials, and in the case of chemical materials, all analogs, formulations, mixtures and compositions of those materials. Research Materials are the sole property of the Company. Consultant

agrees not to use or evaluate Research Materials for any purpose other than as directed by the Company, and not to transfer the Research Materials to any third party without the prior written consent of the Company. Consultant will use the Research Materials in strict compliance with all laws and regulations.

3.5 **Records.** Consultant shall make and maintain adequate and current written records of all Work Product, which records shall be available to and remain the property of the Company at all times.

3.6 **Agreement with Institution**. This Agreement is made subject to the understanding that Consultant, if affiliated with an Institution, may be required to fulfill certain obligations, including teaching, directing laboratory operations, conducting research, and publishing work. It is further understood that Consultant may have signed an agreement concerning inventions with Institution, under which Consultant may be obligated to assign to Institution certain inventions which arise out of or otherwise relate to Consultant's work at or for Institution or from Consultant's use of certain of its facilities or intellectual property. In performing the Consulting Services, Consultant agrees not to utilize Institution facilities or intellectual property if the result of such use is that any Inventions will not be assignable solely to the Company. Use of Institution's telephone, fax machines or computers for communication purposes, however, will not constitute use of Institution's facilities under this Agreement.

3.7 **Work at Third Party Facilities.** Consultant agrees not to make use of any funds, space, personnel, facilities, equipment or other resources of a third party in performing the Consulting Services, and further agrees not to take any other action that would result in a third party owning or having a right in any Inventions, unless agreed upon in writing in advance by the Company.

4. **Confidential Information**.

4.1 **Definition.** "Confidential Information" means information with respect to the facilities and methods of the Company, Research Materials, trade secrets, Inventions, systems, patents and patent applications, procedures, manuals, confidential reports, financial or legal information, business plans, prospects, or opportunities, personnel information, lists of customers and suppliers, and information of third parties provided by the Company to Consultant. Confidential Information does not include information which (i) is in the public domain or which becomes part of the public domain through no wrongful act on Consultant's part but only after it becomes so publicly known, (ii) is already in Consultant's possession at the time of disclosure by the Company, other than by previous disclosure by the Company, as evidenced by written or electronic records, or (iii) that becomes known to Consultant through disclosure by a third party having the right to disclose the information, as evidenced by written or electronic records.

4.2 **Obligations of Confidentiality**. Consultant will not directly or indirectly publish, disseminate or otherwise disclose, use for Consultant's own benefit or for the benefit of a third party, deliver or make available to any third party, any Confidential Information, other than in furtherance of the purposes of this Agreement, and only then with the prior written consent of the Company, and it is agreed and understood that all Confidential Information shall remain the sole property of the Company. Without the Company's prior written approval, Consultant will not directly or indirectly disclose to anyone the existence or terms of this Agreement or the fact that Consultant has this arrangement with the Company. If required, Consultant may disclose the Confidential Information to a governmental

authority or by order of a court of competent jurisdiction, provided that such disclosure is subject to all applicable governmental or judicial protection available for like material and reasonable advance notice of such compulsory disclosure is given to the Company. Consultant will exercise all reasonable precautions to protect the physical integrity and confidentiality of the Confidential Information, and will not remove any Confidential Information or copies or derivations thereof from the Company's premises except to the extent necessary to fulfill the Consulting Services, and then only with the Company's prior consent. Consultant may disseminate or permit access to Confidential Information only to Consultant Personnel who have a need to know such Confidential Information in the course of the performance of their duties under this Agreement and who are bound to protect the confidentiality of the Confidential Information consistent with the terms of this Agreement. Consultant agrees to be responsible for any breach of this Agreement by any of the Consultant Personnel. The Company will be entitled to injunctive relief as a remedy for any breach of the terms of this Section 4. Nothing in this Agreement limits, restricts or in any other way affects Consultant's communicating with any governmental agency or entity, or communicating with any official or staff person of a governmental agency or entity, concerning matters relevant to the governmental agency or entity, or requires Consultant to furnish prior notice to the Company of the same. Consultant cannot be held criminally or civilly liable under any federal or state trade secret law for disclosing a trade secret (i) in confidence to a federal, state, or local government official, either directly or indirectly, or to an attorney, solely for the purpose of reporting or investigating a suspected violation of law, or (ii) in a complaint or other document filed under seal in a lawsuit or other proceeding. Notwithstanding this immunity from liability, Consultant may be held liable if he unlawfully accesses trade secrets by unauthorized means.

4.3 **Third Party Confidential Information**. Consultant recognizes that the Company has received and in the future will receive from third parties confidential and proprietary information ("Third Party Information") subject to a duty on the Company's part to maintain the confidentiality of such information and to use it only for certain limited purposes. Consultant agrees that Consultant owes the Company and such third parties, during the term of this Agreement and thereafter, a duty to hold Third Party Information in the strictest confidence in accordance with the Company's obligations to such third party, and agrees not to disclose it to any person, firm or corporation or use it except in carrying out the Consulting Services for the Company consistent with the Company's agreement with such third party.

5. Restrictions.

5.1

(i) While Consultant is engaged by the Company, Consultant will not, within the United States or any other geographic region in which the Company conducts its business, and in any capacity, whether individually or as an employee, consultant, director, officer, agent, advisor or otherwise, for or on behalf of any entity (a "Competing Organization"), engage in any business activities that are competitive with any of the material business activities of the Company, including without limitation the research, development, sale or marketing of any competitive product of the Company, unless Consultant's duties at such Competing Organization do not include duties relating to any product, process, service or business activity that competes or is reasonably expected to compete with a material product, process, service or business activity in existence or being conducted, provided or developed by the Company, and provided that Consultant has delivered to the Company a

written statement, confirmed by Consultant's prospective employer or consulting client, as the case may be, describing Consultant's duties and stating that such duties are consistent with Consultant's obligations under this Agreement; and

(ii) While Consultant is engaged by the Company, and for a period of twelve (12) months after the termination or cessation of such engagement for any reason, Consultant will not, whether directly or indirectly, solicit, attempt to solicit or in any manner assist any other party to solicit any employee, independent contractor, or consultant of the Company to terminate or diminish his, her or its relationship with the Company in order to become an employee, consultant, or independent contractor to or for any other person or entity.

5.2 As used in this Section 5, "competitive" activities means discovering, developing or commercializing drugs that selectively target cancer stem cells, "competitive" products means drugs that selectively target cancer stem cells, and an "employee," "independent contractor" or "consultant" of the Company is any person who holds or at any time during the six-month period prior to the termination of Consultant's engagement by Company held such status with Company.

6. Representations and Warranties.

6.1 **No Conflicts**. Consultant is under no contractual or other obligation or restriction which is inconsistent with Consultant's execution of this Agreement or the performance of the Consulting Services. The Company agrees that nothing in this Agreement prohibits or interferes with the Consultant's ability pursue other professional opportunities, except as limited by Sections 3, 4 and 5 above.

6.2 **Absence of Debarment**. Consultant represents that (a) neither Consultant nor any Consultant Personnel has been debarred, and to the best of Consultant's knowledge is not under consideration to be debarred, by the U.S. Food and Drug Administration ("FDA") from working in or providing consulting services to any pharmaceutical or biotechnology company under Section 306(a) or 306(b) of the federal Food, Drug and Cosmetic Act (codified at 21 U.S.C. §§ 335a(a) and 335a(b)); (b) no debarred person will in the future be employed by Consultant to perform any services hereunder in connection with any application for approval of a drug by the FDA; and (c) neither Consultant nor any Consultant Personnel has a conviction on their record for which a person can be debarred as decribed in Sections 306(a) or 306(b) of the federal Food, Drug and Cosmetic Act. Consultant further represents and warrants that should Consultant or any Consultant Personnel be convicted in the future of any act for which a person can be debarred as described in Sections 306(a) or 306(b) of the federal Food, Drug and Cosmetic Act, Consultant shall immediately notify Company of such conviction in writing.

6.3 **Assignment of Ownership in Work Product**. Consultant represents and warrants that (i) Consultant has the right and unrestricted ability to assign the Work Product to the Company as set forth in Section 3 (including without limitation the right to assign any Work Product created by Consultant's employees or contractors); (ii) the Work Product has not heretofore been published in whole or in part; and (iii) the Work Product will not infringe upon any copyright, patent, trademark, right of publicity or privacy, or any other proprietary or intellectual property right of any person, whether contractual, statutory or common law.

6.4 **Compliance with Law**. Consultant covenants that the services to be provided hereunder shall be in compliance with all applicable laws, rules and regulations. Consultant

acknowledges that Consultant is subject to the Company's insider trading policy, a copy of which can be found on the Company's website at www.verastem.com.

6.5 **No Conflicting Agreements.** Consultant represents that Consultant's performance of all the terms of this Agreement and as a provider of services to the Company does not and will not breach any agreement to keep in confidence proprietary information, knowledge or data acquired by Consultant in confidence or in trust prior to or during this Agreement, and Consultant has not and will not disclose to the Company or induce the Company to use any confidential or proprietary information or material belonging to any previous employers or other third parties. When performing the Consulting Services, Consultant agrees to use only such materials and information of any kind that Consultant has rightfully obtained and that are not considered proprietary or confidential by any third party unless agreed to otherwise by the Company in writing.

7. Term and Termination.

7.1 **Term**. This Agreement will commence on the Effective Date and continue for period of three years, that is, until July 21, 2022 ("the Term"). This Agreement is not subject to cancelation by the Company absent cause unless sooner terminated pursuant to the express terms of this Section 7. The Company may, however, during the Term and in its sole discretion, reduce or eliminate the Consulting Services and/or revoke the Consultant's access to Company premises, equipment, property, or systems.

7.2 **Termination for Cause**. If, during the Term, the Consultant (i) materially breaches a material provision of this Agreement which is not remedied within (30) days of written notice thereof, (ii) is convicted of or enters a plea of *nolo contendere* to any felony charge, or (iii) commits acts of fraud, embezzlement or moral turpitude involving the Company, the Company may terminate the Agreement for Cause. In addition, the Company may terminate this Agreement immediately at any time upon written notice to Consultant in the event Consultant revokes his acceptance of the Separation Agreement (as defined below).

7.3 **Termination by Consultant**. Consultant may terminate this Agreement at any time without cause upon not less than thirty (30) days' prior written notice to the Company.

7.4 **Effect of Expiration/Termination.** Upon expiration or termination of this Agreement, neither the Company nor Consultant will have any further obligations under this Agreement, except (a) for liabilities accrued through the date of termination, and (b) the obligations under Sections 3, 4, 5, 6, 7 and 8 hereof will survive. Upon expiration or termination, and in any case upon the Company's request, Consultant will return immediately to the Company all tangible Confidential Information and all tangible Third Party Information, including all copies, reproductions and derivations thereof, and all of the Company's property, equipment, and documents. Consultant will not copy, delete, or alter any information contained on any Company property, equipment, or documents before returning such to the Company. In addition, if Consultant has used any personal computer, server, electronic device, or e-mail system to receive, store, review, prepare or transmit any Confidential Information or Third Party Information, Consultant will provide the Company with a computer-useable copy of all such Confidential Information and Third Party Information and then will delete any such Confidential Information or Third Party Information from Consultant's computer storage or any other media (including, but not limited to, online and off-line libraries). Consultant agrees to provide the Company access to its system as reasonably requested to verify that the necessary copying and/or deletion has been completed. Consultant further agrees that any property situated on Company

premises and owned by the Company will be subject to inspection by the Company's personnel at any time with or without notice. Consultant will, promptly upon expiration or termination, certify in writing that it has complied with the requirements of this section ; provided, however, that Consultants obligations under this Agreement will continue even if Consultants fails or declines to provide such written certification.

8. Miscellaneous.

8.1 Independent Contractor. All Consulting Services will be rendered by Consultant as an independent contractor, and this Agreement does not create an employer-employee, partnership, agency or joint venture relationship between the Company and Consultant. Except as set forth in his Separation Agreement, Consultant will have no rights to receive any employee benefits, such as health and accident insurance, sick leave or vacation which are accorded to regular Company employees, except as may be required by COBRA. Consultant will not in any way represent himself to be an employee, partner, joint venturer, or agent of the Company. Consultant is not authorized to make any representation, contract, or commitment on behalf of the Company or incur any liabilities or obligations of any kind in the name of or on behalf of the Company. Consultant shall work independently, without day-to-day direction from the Company, and may adopt such arrangements as Consultant desires with regard to the details of the Consulting Services performed under this Agreement, the hours during which the Consulting Services will be provided, and the place or places where the Consulting Services are to be furnished; provided that: (a) such arrangements, details, hours and location of services shall be consistent with the proper accomplishment of the agreed objectives of the Company; and (b) such services by Consultant shall be performed in a manner calculated to obtain the most satisfactory results for the Company.

8.2 Taxes. Consultant and the Company agree that the Company will treat Consultant as an independent contractor for purposes of all tax laws (local, state and federal) and file income reporting and other forms consistent with such status. Consultant agrees that, as an independent contractor, neither Consultant nor Consultant's employees are entitled to unemployment benefits in the event this Agreement terminates, or to workers' compensation benefits in the event that Consultant, or any employee of Consultant, is injured in any manner while performing obligations under this Agreement. Consultant will be solely responsible to pay any and all local, state, and/or federal income, social security and unemployment taxes for Consultant and Consultant's employees. The Company will not withhold any taxes or prepare W-2 Forms for Consultant, but will provide Consultant with a Form 1099 if and to the extent required by law. Consultant is solely responsible for, and will timely file, all tax returns and payments required to be filed with, or made to, any federal, state or local tax authority with respect to the performance of services and receipt of fees under this Agreement. Consultant is solely responsible for, and must maintain adequate records of, expenses incurred in the course of performing services under this Agreement, except as provided herein. The Company will regularly report amounts paid to Consultant with the appropriate taxing authorities, as required by law. Consultant will provide the Company with Consultant's taxpayer identification number or social security number, as applicable.

8.3 Use of Name. Consultant consents to the use by the Company of Consultant's name and likeness in written materials and oral presentations to current or prospective customers, partners, investors or others, provided that such materials or presentations accurately describe the nature of Consultant's relationship with and contributions to the Company.

8.4 **Assignability and Binding Effect**. The Consulting Services to be rendered by Consultant are personal in nature. Consultant may not assign or transfer this Agreement or any of Consultant's rights or obligations hereunder except to a corporation of which Consultant is the sole stockholder. In no event will Consultant assign or delegate responsibility for actual performance of the Consulting Services to any other natural person except to Consultant Personnel as provided for under this Agreement. This Agreement will be binding upon and inure to the benefit of the parties and their respective legal representatives, heirs, successors and permitted assigns. The Company may assign this Agreement to any other corporation or entity which acquires (whether by purchase, merger, consolidation or otherwise) all or substantially all of the business and/or assets of the Company.

8.5 **Headings**. The section headings are included solely for convenience of reference and will not control or affect the meaning or interpretation of any of the provisions of this Agreement.

8.6 **Notices**. Any notices or other communications from one party to the other will be in writing and will be given by addressing the same to the other at the address or facsimile number set forth in this Agreement. Notices to the Company will be marked "Attention: General Counsel". Notice will be deemed to have been duly given when (a) deposited in the United States mail with proper postage for first class Registered or Certified Mail prepaid, return receipt requested, (b) sent by any reputable commercial courier, delivery confirmation requested, (c) delivered personally, or (d) if promptly confirmed by mail or commercial courier as provided above, when dispatched by facsimile.

8.7 **Amendment.** This Agreement may be amended or modified only by a writing signed by authorized representatives of both parties.

8.8 **No Waiver.** No waiver of any term or condition of this Agreement shall be valid or binding on either party unless the same shall be been mutually assented to in writing by both parties. The failure of either party to enforce at any time any of the provisions of this Agreement, or the failure to require at any time performance by the other party of any of the provisions of this Agreement, shall in no way be construed to be a present or future waiver of such provisions, nor in any way affect the right of either party to enforce each and every such provision thereafter. The express waiver by either party of any provision, condition or requirement of this Agreement shall not constitute a waiver of any future obligation to comply with such provision, condition or requirement.

8.9 **Severability.** In the event that any one or more of the provisions contained in this Agreement is, for any reason, held to be invalid, illegal or unenforceable in any respect, such invalidity, illegality or unenforceability will not affect any other provisions of this Agreement, and all other provisions will remain in full force and effect. If any provision of this Agreement is held to be excessively broad, it will be reformed and construed by limiting and reducing it so as to be enforceable to the maximum extent permitted by law.

8.10 **Entire Agreement.** This Agreement, together with the Separation Agreement between the parties dated June 25, 2019 (the "Separation Agreement") and the surviving documents referenced therein, constitutes the entire agreement of the parties with regard to their subject matter, and supersede all previous written or oral representations, agreements and understandings between the parties.

8.11 **Governing Law/Jurisdiction**. All disputes related to or arising out of this Agreement shall be resolved in the state or federal courts of the Commonwealth of Massachusetts, to whose exclusive jurisdiction each party hereby consents. This Agreement will be governed by, construed and enforced in accordance with the laws of the Commonwealth of Massachusetts applicable to contracts made and to be performed therein, without giving effect to the principles thereof relating to the conflict of laws.

8.12 **Remedies.** The Parties agree that Consultant's obligations under this Agreement are of a unique character that gives them particular value; breach of any of the provisions of Sections 3, 4 or 5 will result in irreparable and continuing damage to the Company for which there will be no adequate remedy at law; and, in the event of such breach or threatened breach, the Company will be entitled to injunctive relief and/or a decree for specific performance, an award of its attorney's fees incurred, and such other and further relief as may be proper. Consultant and the Company further agree that no bond or other security shall be required in obtaining such equitable relief.

8.13 **Counterparts**. This Agreement may be executed in any number of counterparts, each of which will be deemed an original, but all of which together will constitute one and the same instrument.

IN WITNESS WHEREOF, the parties hereto have duly executed this Agreement under seal as of the Effective Date.

VERASTEM, INC.

By: /s/ Michael Kauffman MD
Name: Michael Kauffman
Title: Lead Director, VSTM BoD
duly authorized

CONSULTANT

By: /s/ Robert Forrester
Name: Robert Forrester
Title:
duly authorized

INSTITUTION ACKNOWLEDGEMENT
AND CONSENT FORM

Verastem, Inc. (the "Company") is prepared to enter into the foregoing Agreement with the consultant named on the preceding signature page ("Consultant"). The Company recognizes that as a member of the institution named below ("Institution"), Consultant is responsible for ensuring that any consulting agreement Consultant enters into with a for-profit entity is not in conflict with the patent, consulting or other policies of Institution. The proposed Agreement requires Consultant, if required by Institution policies, to disclose the proposed Agreement to Institution and/or to obtain Institution's consent to enter into the proposed Agreement.

Institution hereby acknowledges and consents to Consultant entering into the foregoing Agreement.

INSTITUTION:

By_____

Print Name

Title_____
duly authorized

Date_____

June 25, 2019

Robert Forrester
346 Gay Street
Westwood, MA 02142

Dear Robert:

As we have agreed, you have resigned your employment with Verastem, Inc. (the "Company") and your position on the Verastem Board of Directors, and the Company has accepted that resignation, effective June 21, 2019 (the "Separation Date"). Any capitalized term that is not defined herein shall have the same meaning as defined in the Amended and Restated Employment Agreement between you and the Company dated as of November 22, 2013 (the "Employment Agreement"). The purpose of this letter (the "Agreement") is to confirm the terms concerning your separation from employment, as follows:

1. Final Salary and Vacation Pay. In signing this Agreement, you acknowledge that you have received pay for all work you have performed for the Company through the Separation Date, to the extent not previously paid. Since, in accordance with Company policy, you have no accrued vacation days as of the Separation Date, you will not receive any pay for such vacation time. You will receive the payments described in this Section 1 regardless of whether or not you elect to sign this Agreement.

2. Severance Benefits. In consideration of your acceptance of this Agreement and subject to your meeting in full your obligations hereunder and under the Confidentiality Agreement (as defined below), and in full satisfaction of any rights that you have under the Employment Agreement, the Company will provide you with the following severance benefits:

a. The Company will pay you your salary, at your final base rate of pay, for a period of thirteen (13) months following the Separation Date. Payments will be made in the form of salary continuation, and will begin on the Payment Commencement Date. The first payment will be retroactive to the day immediately following the Separation Date.

b. If you are enrolled in the Company's group medical and/or dental plans on the Separation Date, you may elect to continue your participation and that of your eligible dependents in those plans for a period of time under the federal law known as "COBRA," or similar applicable state law (together, "COBRA"). You may make such an election whether or not you accept this Agreement. However, if you accept this Agreement and you timely elect to continue your participation and that of your eligible dependents in the plans, the Company will pay or, at its option, reimburse you for the full premium cost of that participation until the earlier of the conclusion of thirteen (13) months following the Separation Date or the date that you

become eligible to enroll in the health (or, if applicable, dental) plan of a new employer. Payments will begin on the Payment Commencement Date. The first payment will be retroactive to the day immediately following the Separation Date. If the Company's contributions end before your entitlement to coverage under COBRA concludes, you may continue such coverage by paying the full premium cost yourself. Notwithstanding the foregoing, in the event that the Company's payment of the COBRA premium amounts, as described under this Section 2(b), would subject the Company to any tax or penalty under the Patient Protection and Affordable Care Act (as amended from time to time, the "ACA") or Section 105(h) of the Internal Revenue Code of 1986, as amended ("Section 105(h)"), or applicable regulations or guidance issued under the ACA or Section 105(h), or any other applicable law, in each case, it shall gross up the such payments' tax consequences, if any, to you. Notwithstanding the foregoing, if the payment or reimbursement by the Company of the premium costs, including payment of any gross up for any tax consequences, described in the preceding sentence, will subject or expose the Company to taxes or penalties, you and the Company agree to work together in good faith efforts to renegotiate the provisions of this section and to enter into a substitute arrangement pursuant to which the Company may not be subjected or exposed to taxes or penalties but which will not adversely affect the full economic value to you of the benefits promised by this provision. For avoidance of any doubt, nothing in this provision will require you to accept any renegotiated agreement that would disadvantage you economically or required that you accept a lesser quality of health care coverage for yourself or your family.

3. Acknowledgement of Full Payment and Withholding.

a. You acknowledge and agree that the payments provided under Section 1 of this Agreement are in complete satisfaction of any and all compensation and benefits due to you from the Company, whether for services provided to the Company or otherwise, through the Separation Date, except as expressed provided herein and that, except as expressly provided under this Agreement, no further compensation or benefits are owed or will be paid or provided to you.

b. All payments made by the Company under this Agreement shall be reduced by any tax or other amounts required to be withheld by the Company under applicable law and all other lawful deductions authorized by you.

4. Status of Employee Benefits, Paid Time Off, Expenses and Equity.

a. Except as otherwise provided in Section 2 or required by applicable law, your participation in all employee benefit plans of the Company will end as of the Separation Date, in accordance with the terms of those plans. You will not continue to earn vacation, paid time off or other similar benefits after the Separation Date or be reimbursed for any expenses incurred after the Separation Date that have not otherwise been approved in writing by the Company. You will receive information about your COBRA continuation rights under separate cover.

b. You agree that, within four (4) weeks immediately following the Effective Date (as defined below), you will submit your final expense reimbursement statement reflecting all business expenses you incurred through the Separation Date, if any, for which you seek reimbursement, and, in accordance with Company policy, reasonable substantiation and documentation for the

same. The Company will reimburse you for your authorized and documented expenses pursuant to its regular business practice.

c. All unvested stock options granted by the Company to you prior to the Separation Date which as of the Separation Date are outstanding and, by their terms, vest only based on the passage of time and which would have vested during the twenty-five (25) month period immediately following the Separation Date, shall, notwithstanding the terms of the stock or equity compensation plans and award agreements to which such stock options are subject, automatically become fully vested as of the Effective Date, on the condition that you have timely executed this Agreement and it remains effective on the Effective Date. In addition, all other unvested stock options granted by the Company to you prior to the Separation Date, the vesting of which are based on individual or Company performance and that are outstanding as of the Separation Date shall, notwithstanding the terms of the stock or equity compensation plans and award agreements to which such stock options are subject, for the twenty-five (25) month period immediately following the Separation Date, remain outstanding and eligible to vest based on the achievement of the individual or Company performance metrics to which such awards are subject, in each case subject to the condition that you have timely executed this Agreement and it remains effective on the Effective Date. For the avoidance of doubt, all stock options held by you that have not vested as of or on July 21, 2021 shall automatically terminate and be forfeited without further action on the part of the Company. All stock options that have vested as of or on July 21, 2021 shall, notwithstanding the terms of the stock or equity compensation plans and any applicable award agreements to which such stock options are subject, remain exercisable through July 21, 2022, and shall automatically terminate and be forfeited without further action on the part of the Company if such stock options have not been exercised on or before July 21, 2022.

d. In the event of any conflict between the vesting provisions applicable to the equity-based awards set forth in this Section and the terms of the sock or equity compensation plans and any applicable award agreements, the provisions of this Section shall govern.

e. Within ten (10) business days of the complete execution of this Agreement, the Company shall reimburse you for reasonable legal expenses incurred or paid by you in connection with providing advice to you concerning the conclusion of your employment with the Company, including but not limited to the drafting and execution of this Agreement and any other Agreement(s) concerning your continued provision of services to the Company following the Separation Date, up to a maximum of twelve thousand dollars ($12,000).

f. You and the Company are contemporaneously entering into a separate consulting agreement, setting forth the terms of your continued service to the Company as an independent contractor immediately following the Separation Date (the "Consulting Agreement"). Except as expressly provided herein or in the Consulting Agreement, and notwithstanding anything to the contrary set forth in the Company's Amended and Restated 2012 Incentive Plan or any award agreement between you and the Company thereunder, all stock options granted to you will cease to vest as of the Separation Date, and any unvested stock options shall be forfeited.

5. Confidentiality, Non-Disparagement, and Continuing Obligations.

a. You acknowledge and agree that you will continue to comply with your obligations under the Employee Non-Solicitation, Non-Competition, Confidential Information and Inventions Assignment Agreement between you and the Company dated as of October 7, 2014 (the "Confidentiality Agreement") that survive the termination of your employment by necessary implication or the terms thereof.

b. Subject to Section 7(b) of this Agreement and except as may be required by applicable law or legal process, you agree that you will not disparage or criticize the Company, its Affiliates, their business, their management or their products or services, and that you will not otherwise do or say anything that could reasonably be expected to disrupt the good morale of employees of the Company. The Company agrees that, by and through its Board of Directors and officers, it shall not disparage or criticize you nor will it do or say anything that could reasonably be expected to damage your personal or professional reputation or interests. Additionally, for the avoidance of all doubt, nothing in this Section prohibits the Parties from responding candidly as required by law.

6. Return of Company Documents and Other Property. Except as may be agreed is necessary given your ongoing consulting relationship, in signing this Agreement you represent and warrant that you have returned to the Company any and all documents, materials and information (whether in hardcopy, on electronic media or otherwise) that is proprietary and related to the business of the Company and its Affiliates (whether present or otherwise), and all keys, access cards, credit cards, computer hardware and software, telephones and telephone-related equipment and all other property of the Company or any of its Affiliates in your possession or control. The Company agrees that, notwithstanding the foregoing, you may retain your computer, and to the extent the Company may have assisted in the purchase of your cell phones, your cell phone(s); provided that, you represent that you have already deleted or shall promptly delete from the computer's and cell phone's hard drive or files any Company proprietary information. Further, you represent and warrant that you have not retained any copy or derivation of any documents, materials or information (whether in hardcopy, on electronic media or otherwise) of the Company or any of its Affiliates. You also agree that if you should later discover any document or other property of the Company or any of its Affiliates in your possession, you will immediately return it to the Company and delete it from your possession. You agree that you will not, following the Separation Date, for any purpose, attempt to access or use any computer or computer network or system of the Company or any of its Affiliates, including without limitation the electronic mail system. Further, you acknowledge that you have disclosed to the Company all passwords necessary or desirable to obtain access to, or that would assist in obtaining access to, all information which you have password-protected on any computer equipment, network or system of the Company or any of its Affiliates. It is, however, understood and agreed that you may retain possession of and access to certain Company property, equipment, and documents as expressly directed by the Company in order to fulfill your obligations under the Consulting Agreement.

7. General Release and Waiver of Claims.

a. In exchange for and in consideration of the special severance pay and other benefits provided to you under this Agreement, which are conditioned on your signing of this Agreement,

to which you would not otherwise be entitled, and other good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged, on your own behalf and that of your heirs, executors, and beneficiaries, and all others claiming through you, you agree that this Agreement shall be in complete and final settlement of any and all causes of action, rights and claims, whether known or unknown, that you have had in the past, now have, or might now have, in any way related to, connected with or arising out of your employment or your other association with the Company or any of its Affiliates or the termination of the same or pursuant to Title VII of the Civil Rights Act, the Americans with Disabilities Act, the Age Discrimination in Employment Act, as amended by the Older Workers Benefit Protection Act, the Employee Retirement Income Security Act, the wage and hour, wage payment and/or fair employment practices laws and statutes of the state or states in which you have provided services to the Company or any of its Affiliates (each as amended from time to time), and/or any other federal, state or local law, regulation or other requirement, and you hereby release and forever discharge the Company, its subsidiaries and other Affiliates and all of their respective past, present and future directors, shareholders, officers, members, managers, investors, joint venturers, general and limited partners, employees, employee benefit plans, administrators, trustees, agents, representatives, predecessors, successors and assigns, and all others connected with any of them, both individually and in their official capacities, to the extent that each and any individual has an affiliation with the Company that is known to you, from any and all such causes of action, rights and claims.

b. Notwithstanding the foregoing, nothing contained in this Agreement shall be construed to prohibit you from filing a charge with or participating in any investigation or proceeding conducted by the federal Equal Employment Opportunity Commission or a comparable state or local agency, except that you hereby agree to waive your right to recover monetary damages or other individual relief in any such charge, investigation or proceeding, or any related complaint or lawsuit filed by you or anyone else on your behalf. Further, nothing contained in this Agreement or the Confidentiality Agreement limits, restricts or in any other way affects your communicating with any governmental agency or entity, or communicating with any official or staff person of a governmental agency or entity, concerning matters relevant to the governmental agency or entity. Additionally, nothing in this Release shall limit or otherwise restrict your rights as follows: (i) to bring claims for indemnification in your capacity as an officer or director of the Company under the Company's Certificate of Incorporation, Bylaws or agreement, if any, providing for director or officer indemnification; (ii) to receive insurance payments under any policy maintained by the Company that would otherwise apply to you; (iii) to receive retirement benefits that are accrued and fully vested as of your Separation Date under such plans protected by ERISA. Additionally, this release shall not prevent you from defending yourself against a released party in the event that any such released party initiates legal action against you pertaining to events or circumstances arising prior to the effective date of this release (other than a legal action that solely alleges claims involving or arising out of a violation of law that would constitute a felony or other crime involving moral turpitude by you that amounts to criminal misconduct on your part). In such a circumstance, this release shall be null and void as against the party initiating litigation alone, and you may bring any counter-claim you may have against that party.

c. In signing this Agreement, you acknowledge your understanding that you may consider the terms of this Agreement for up to twenty-one (21) days from the date you receive it. You also acknowledge that you are hereby advised by the Company to seek the advice of an

attorney prior to signing this Agreement; that you have had sufficient time to consider this Agreement and to consult with an attorney, if you wished to do so, or to consult with any other person of your choosing before signing; and that you are signing this Agreement voluntarily and with a full understanding of its terms.

d. You further acknowledge that, in signing this Agreement, you have not relied on any promises or representations, express or implied, that are not set forth expressly in this Agreement. You understand that you may revoke this Agreement at any time within seven (7) days of the date of your signing by written notice to the Lead Director of the Company's Board of Directors and that this Agreement will take effect only upon the expiration of such seven-day revocation period and only if you have not timely revoked it (the "Effective Date").

8. Miscellaneous.

a. This Agreement, together with the Consulting Agreement, constitutes the entire agreement between you and the Company and its Affiliates and supersedes all prior and contemporaneous communications, agreements and understandings, whether written or oral, with respect to your employment, its termination and all related matters, excluding only the Confidentiality Agreement, which shall remain in full force and effect in accordance with its terms, and your rights and obligations with respect to your vested options.

b. This Agreement may not be modified or amended, and no breach shall be deemed to be waived, unless agreed to in writing by you and an authorized representative of the Company. The captions and headings in this Agreement are for convenience only, and in no way define or describe the scope or content of any provision of this Agreement.

c. The obligation of the Company to make payments to you or on your behalf under this Agreement, and your right to retain the same, is expressly conditioned upon your continued full performance of your obligations under this Agreement and the Confidentiality Agreement.

d. This is a Massachusetts contract and shall be governed and construed in accordance with the laws of the Commonwealth of Massachusetts, without regard to any conflict of laws principles that would result in the application of the laws of another jurisdiction. You agree to submit to the exclusive jurisdiction of the courts of and in the Commonwealth of Massachusetts in connection with any dispute arising out of this Agreement.

[Remainder of Page Intentionally Left Blank]

If the terms of this Agreement are acceptable to you, please sign, date and return it to Cathy Carew within twenty-one (21) days of the date you receive it. You may revoke this Agreement at any time during the seven-day period immediately following the date of your signing by notifying the Lead Director of the Company's Board of Directors in writing of your revocation within that period. If you do not revoke this Agreement, then, on the eighth day following the date that you signed it, this Agreement shall take effect as a legally binding agreement between you and the Company on the basis set forth above. You agree that if there have been any changes to a prior version of this Agreement (material or immaterial), the 21 day consideration period will not be reset. The enclosed copy of this letter, which you should also sign and date, is for your records.

Sincerely,
Verastem, Inc.

By: /s/ Dr. Michael Kauffman
Dr. Michael Kauffman
Lead Director

Accepted and agreed:

Signature: /s/ Robert Forrester
Robert Forrester

Date: June 25, 2019

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CONSULTING AGREEMENT

This Consulting Agreement dated as of June 27, 2019 (this "Agreement") defines the terms upon which Verastem, Inc. (hereinafter referred to as "Verastem") has agreed to engage the consulting services of Joseph Lobacki ("Consultant").

1. **Consulting Services.** Consultant shall provide consulting services to Verastem in the field all as more particularly described on Exhibit A (the "Engagement"). Robert Gagnon, of Verastem (the "Verastem Contact"), shall coordinate all consulting activities to be performed under this Agreement. Please send all reports and other communications to the Verastem Contact.
Verastem may designate a different Verastem Contact by written notice to Consultant.

   a. **Time Commitment.** Consulting services will be provided to Verastem from time to time upon Verastem's request. Consultant is expected to be available to Verastem personnel by phone, email or facsimile.

1. **Term.** This consulting engagement shall commence on June 29, 2019 and shall continue until January 15, 2020, unless extended by mutual written consent or sooner terminated as provided below (the "Consultation Period"). Either party to this Agreement may terminate this consulting engagement upon 30 days prior written notice to the other party, for any reason and without prejudice to any right or remedy Consultant may have due to any failure of the other party to perform Consultant's obligations under this Agreement. In the event of such termination, Consultant shall be entitled to payment for services performed and expenses paid or incurred prior to the effective date of termination. All obligations under this Agreement, which by their nature are intended to continue beyond the termination of the Consultation Period, shall survive a termination by either or both parties of the Consultation Period.

2. Compensation.

   a. **Consulting Fees**. Verastem shall pay Consultant $350.00 per hour for each hour spent, up to a maximum of $220,000 over the term of the Agreement, by the Consultant in the performance of consulting, advisory or related services for Verastem, payable within 45 days of the end of each month upon presentation of a Verastem approved invoice by Consultant fully detailing the time spent and tasks performed by Consultant. Invoices should reference this Agreement and should be submitted to: Accounts Payable, Verastem, Inc., 117 Kendrick Street, Suite 500, Needham, MA 02494 or via email at ap@verastem.com.

   b. **Reimbursement of Expenses**. Verastem will pay for any reasonable travel, meals and lodging expenses associated with the performance of consulting services hereunder upon receipt of appropriate and complete documentation on Verastem approved invoice forms. Verastem may also provide or directly pay vendors to provide you with reasonable travel, lodging, and meals when you are providing services hereunder.

3. Confidential Information; Inventions and Patent Filings.

   a. **Confidential Information**. Consultant acknowledges that Consultant's relationship with Verastem is one of high trust and confidence and that in the course of Consultant's service to Verastem Consultant will have access to and contact with Confidential Information (as defined below). Consultant will not, during the Consultation Period or at any time within two years thereafter, disclose to others (including other third parties already under an obligation of

confidentiality to Verastem), or use for Consultant's benefit or the benefit of others, any Confidential Information or Invention (as defined below). Without limiting the foregoing, you agree that you shall not trade any securities of Verastem based upon any confidential information learned pursuant hereto.

I. For purposes of this Agreement, Confidential Information shall mean all information (whether or not patentable or copyrightable) owned, possessed or used by Verastem, including, without limitation, any Invention, formula, trade secret, process, prototype, device, equipment, research, report, technical data, know-how, technology and marketing or business plan, that is communicated to, learned of, developed or otherwise acquired by Consultant in the course of performing consulting services for Verastem hereunder.

II. Consultant's obligations under this Section shall not apply to any information that Consultant demonstrates (i) is or becomes known to the general public under circumstances involving no breach by Consultant or others of the terms of this Section, or (ii) is in the Consultant's possession at the time of disclosure otherwise than as a result of a prior disclosure by Verastem to Consultant or by a third party to Consultant where that third party is or was under an obligation of confidentiality with respect thereto. In addition, Consultant will not be liable to Verastem for trade secret misappropriation if Consultant discloses a trade secret (a) in confidence to a federal, state, or local government official, or to an attorney, solely for the purpose of reporting or investigating a suspected violation of law and (b) in any document filed under seal in a lawsuit or other proceeding.

III. Consultant represents that Consultant's engagement as a consultant with Verastem and performance under this Agreement does not, and shall not, breach any agreement that obligates Consultant to keep in confidence any trade secrets or confidential or proprietary information of Consultant or of any other party or to refrain from competing, directly or indirectly, with the business of any other party. Consultant shall not disclose to Verastem any trade secrets or confidential or proprietary information of any other party.

b. Inventions. All inventions, discoveries, data, technology, designs, innovations and improvements related to the Engagement or business of Verastem (including without limitation all inventions and improvements relating to Verastem prototypes or products) which are made, conceived or reduced to practice by Consultant, solely or jointly with others, during the Consultation Period shall be the sole property of Verastem. In addition, all inventions, discoveries, data, technology, designs, innovations and improvements which are made, conceived or reduced to practice by Consultant, solely or jointly with others, during the Consultation Period or thereafter, shall be the sole property of Verastem, if resulting or derived from Confidential Information (the inventions referenced in the first two sentences being hereinafter referred to as "Inventions"). Consultant hereby assigns to Verastem all rights Consultant has in Inventions and any and all related patents, copyrights, trademarks, trade names, and other industrial and intellectual property rights and applications therefor, in the United States and elsewhere and appoints any officer of Verastem as Consultant's duly authorized attorney to execute, file, prosecute and protect the same before any government agency, court or authority. Upon the request of Verastem and at Verastem's expense, Consultant shall execute such further assignments, documents and other instruments as may be necessary or desirable to fully and completely assign all Inventions to Verastem and to assist Verastem in applying for, obtaining and enforcing patents or copyrights or other rights in the United States and in any foreign country with respect to any Invention. Consultant shall promptly disclose all relevant Inventions to Verastem in writing.

c. **Patent Filings.** In order to preserve Verastem's rights under this Agreement, during the Consultation Period and for two (2) years thereafter, Consultant shall submit to Verastem for review, on a confidential basis, any relevant patent applications naming Consultant or its agents, employees or representatives as an inventor, either alone or with others, which Consultant or any third party intends to file with any U.S. or international patent offices. Such applications must be submitted to Verastem at least 30 days in advance of any filing with a U.S. or international patent office. If Verastem determines in good faith, within this 30-day period, that the filing of such an application would be contrary to its intellectual property rights under this agreement, Consultant will amend the proposed patent application to remove any language that is determined by Verastem to be contrary to its intellectual property rights.

d. **Third Party Information.** Consultant understands that Verastem has received and in the future will receive from third parties confidential or proprietary information ("Third Party Information") subject to a duty on Verastem's part to maintain the confidentiality of such information and to use it only for certain limited purposes. During Consultation Period and thereafter, Consultant will hold Third Party Information in the strictest confidence and will not disclose to anyone (other than Verastem personnel who need to know such information in connection with their work for Verastem) or use, except in connection with Consultant's work for Verastem, Third Party Information unless expressly authorized by an officer of Verastem in writing.

e. **No Improper Use of Information of Prior Employers and Others.** During the Consultation Period, Consultant will not improperly use or disclose any confidential information or trade secrets, if any, of any former employer or any other person or entity to whom Consultant has an obligation of confidentiality, and Consultant will not bring onto the premises of Verastem any unpublished documents or any property belonging to any former employer or any other person or entity to whom Consultant has an obligation of confidentiality unless consented to in writing by that former employer or person. Consultant will use, in the performance of Consultant's duties only, information which is generally known and used by persons with training and experience comparable to Consultant's own, which is common knowledge in the industry or otherwise legally in the public domain, or which is otherwise provided or developed by Verastem.

4. **Company Property.** All materials and property provided to Consultant (including all samples, equipment, prototypes, devices, reports, communications, drawings, notes, and analyses), or produced by Consultant, in connection with the performance of services under this Agreement shall be the exclusive property of Verastem. Consultant will keep in Consultant's custody and control any such materials or property that Consultant receives or develops, and will return the same to Verastem upon the termination of the Consultation Period or at Verastem's request. Consultant agrees to treat such materials and property with at least the same degree of confidentiality and care that it keeps Consultant's own materials and property. No materials containing proprietary information may be thrown in the trash unless the same are shredded.

5. **Use of Name.** Verastem may identify Consultant to third parties and in written literature as a consultant to Verastem.

6. **Independent Contractor Status.** Consultant shall perform all services under this Agreement as an "independent contractor" and not as an employee or agent of Verastem. Consultant is not authorized to assume or create any obligation or responsibility, express or implied, on behalf of, or in the name of, Verastem or to bind Verastem in any manner.

7. **Representations and Warranties.** Consultant represents and warrants that Consultant has no duty or obligation to any third party with respect to the Engagement. Consultant further represents and warrants that the services covered by this Agreement are not in violation of any other agreement or obligation by which Consultant is bound.

8. **Release and Indemnification**. In no event shall Consultant be liable to the Company or any other party, whether a claim be in tort, contract or otherwise, for any consequential, indirect, lost profit or similar damages relating the services provided under this Agreement.

9. **Notice.** All notices required or permitted under this Agreement shall be in writing and shall be deemed effective upon personal delivery or upon deposit in the United States Post Office, by registered or certified mail, postage prepaid, addressed:

If to Verastem:
Verastem, Inc.
117 Kendrick Street, Suite 500
Needham, MA 02494

Attn: Verastem Contact,
With a copy to Verastem General Counsel

If to Consultant:
Joseph Lobacki
55 Commercial Wharf, Unit 8
Boston, MA 02110

10. **Miscellaneous.** This Agreement constitutes the entire agreement between the parties and supersedes all prior agreements and understandings, whether written or oral, relating to the subject matter of this Agreement. This Agreement may be amended or modified only by a written instrument executed by both Verastem and the Consultant. This Agreement shall be construed, interpreted and enforced in accordance with the laws of the Commonwealth of Massachusetts. This Agreement shall be binding upon, and inure to the benefit of, both parties and their respective successors and assigns, including any corporation with which, or into which, Verastem may be merged or which may succeed to its assets or business, provided, however, that the obligations of the Consultant are personal and shall not be assigned by Consultant. If any of the provisions of this Agreement is found to be unenforceable or prohibited by any applicable law, such provision shall be ineffective only to the extent of such unenforceability or prohibition without invalidating the remainder of such provision or the remaining provisions of this Agreement.

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IN WITNESS WHEREOF, the parties hereto have executed this Agreement as of the day and year set forth above.

VERASTEM, INC.

By: /s/ Peter Pellegrino
Name: Peter Pellegrino
Title: Vice President, Corporate Controller & Treasurer
duly authorized

CONSULTANT

By: /s/ Joseph Lobacki
Name: Joseph Lobacki
Title:
duly authorized

Exhibit A

Consultant will provide ad hoc projects related to the commercialization of COPIKTRA, as may be requested from time to time by the Company and under the direction of the Company's management.

Exhibit 21.1

List of Registrant's Subsidiaries

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

Verastem Europe GmbH, incorporated in Germany, a wholly owned subsidiary.

Exhibit 23.1

Consent of Independent Registered Public Accounting Firm

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

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

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

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

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

(5) Registration Statement (Form S-8 No. 333-211235) pertaining to the 2012 Incentive Plan of Verastem, Inc.,

(6) Registration Statement (Form S-8 No. 333-218768) pertaining to the 2014 Inducement Award Program of Verastem, Inc.,

(7) Registration Statement (Form S-8 No. 333-218769) pertaining to the 2012 Incentive Plan of Verastem, Inc.;

(8) Registration Statement (Form S-8 No.333-223616) pertaining to the 2014 Inducement Award Program of Verastem, Inc.,

(9) Registration Statement (Form S-3 No. 333-226322) of Verastem, Inc.,

(10) Registration Statement (Form S-8 No.333-228309) pertaining to the 2014 Inducement Award Program of Verastem, Inc. and,

(11) Registration Statement (Form S-8 No.333-229430) pertaining to the 2018 Employee Stock Purchase Plan, 2012 Amended and Restated Incentive Plan, and 2014 Inducement Award Program of Verastem, Inc.

of our reports dated March 11, 2020, with respect to the consolidated financial statements of Verastem, Inc. and the effectiveness of internal control over financial reporting of Verastem, Inc., included in this Annual Report (Form 10-K) of Verastem, Inc. for the year ended December 31, 2019.

/s/Ernst & Young LLP

Boston, Massachusetts
March 11, 2020

Exhibit 31.1

CERTIFICATIONS

I, Brian M. Stuglik certify that:

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

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

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

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

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

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

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

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

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

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

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

/s/ BRIAN M. STUGLIK
Brian M. Stuglik
Chief Executive Officer

Date: March 11, 2020

Exhibit 31.2

CERTIFICATIONS

I, Robert Gagnon, certify that:

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

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

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

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

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

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

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

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

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

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

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

/s/ ROBERT GAGNON
Robert Gagnon
Chief Business and Financial Officer

Date: March 11, 2020

Exhibit 32.1

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

In connection with the Annual Report on Form 10-K of Verastem, Inc. (the "Company") for the period ended December 31, 2019 as filed with the Securities and Exchange Commission (the "SEC") on the date hereof (the "Report"), the undersigned, Brian M. Stuglik, Chief Executive Officer of the Company, hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to my knowledge:

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

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

/s/ BRIAN M. STUGLIK
Brian M. Stuglik
Chief Executive Officer

Date: March 11, 2020

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

Exhibit 32.2

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

In connection with the Annual Report on Form 10-K of Verastem, Inc. (the "Company") for the period ended December 31, 2019 as filed with the Securities and Exchange Commission (the "SEC") on the date hereof (the "Report"), the undersigned, Robert Gagnon, Chief Business and Financial Officer of the Company, hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to my knowledge:

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

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

/s/ ROBERT GAGNON
Robert Gagnon
Chief Business and Financial Officer

Date: March 11, 2020

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

Verastem Oncology Reports Fourth Quarter and Full-Year 2019 Financial Results

BOSTON, MA – March 11, 2020 – Verastem, Inc. (Nasdaq:VSTM) (also known as Verastem Oncology), a biopharmaceutical company committed to developing and commercializing new medicines for patients battling cancer, today reported financial results for the three months and full-year ended December 31, 2019, and provided an overview of recent corporate highlights.

Brian Stuglik, Chief Executive Officer of Verastem Oncology, commented, "We are very excited to be executing on our new strategic direction. Our newly expanded development pipeline and priorities, combined with our recently strengthened balance sheet, leave us well positioned to deliver on our key corporate objectives in 2020 and beyond."

New Strategic Direction

CH5126766 (VS-6766) in Combination with Defactinib

- ***Accelerating Development for KRAS Mutant Solid Tumors.*** In early 2020, Verastem Oncology licensed exclusive global development and commercialization rights to CH5126766 (VS-6766), a unique and promising inhibitor of the RAF/MEK signaling pathway. The combination of CH5126766 (VS-6766) and defactinib is currently being investigated in a Phase 1 clinical study and expansion cohorts in patients with KRAS mutant advanced solid tumors, including low grade serous ovarian cancer, non-small cell lung cancer and colorectal cancer. Verastem Oncology plans to initiate discussions with regulatory authorities during the first half of 2020, with the goal of commencing a registration-directed trial as soon as possible.

  Data from the Phase 1 combination study were submitted for presentation to the American Association for Cancer Research (AACR) 2020 Annual Meeting. The AACR recently announced that it was terminating the April 2020 meeting due to the COVID-19 outbreak and is planning to reschedule the meeting for later this year. Verastem Oncology is actively working with the appropriate organizations and institutions to determine next steps.

Duvelisib (COPIKTRA®)

- ***Prioritizing the Advancement of Duvelisib in Relapsed/Refractory PTCL.*** At the American Society of Hematology 2019 Annual Meeting, Verastem Oncology presented positive data from the dose optimization portion of the Phase 2 PRIMO study evaluating duvelisib in patients with relapsed or refractory PTCL, an aggressive disease with a lack of effective therapeutic options. This initial phase of the trial demonstrated promising clinical activity including complete and durable responses, as assessed by independent central review, with a manageable safety profile. The expansion phase of this registration-directed study continues to accrue patients and Verastem Oncology expects to complete enrollment in 2020 and report top-line results from the expansion cohorts in early 2021. Verastem Oncology intends to build on the existing Fast Track and Orphan Drug Designations and submit a regulatory package to the U.S. Food and Drug Administration to expand the approved indications for COPIKTRA to include relapsed or refractory PTCL.

- ***Focusing COPIKTRA Commercial Activities.*** Verastem Oncology will be reducing the resources directed to the promotion and sale of COPIKTRA in its current indications, including reducing the size of its

salesforce and non-core clinical research. The Company plans to shift its COPIKTRA promotional resources toward large, community-based practices and academic institutions, which represent the majority of the appropriate third-line patients with chronic lymphocytic leukemia/small lymphocytic lymphoma and follicular lymphoma. The Company expects to reduce its overall headcount number to approximately 90 employees.

Corporate and Financial

- ***Strengthened the Balance Sheet Through a Private Placement with Premier Life Science Investors.*** On March 3, 2020, Verastem Oncology completed a private placement offering of approximately 46.5 million shares of its common stock to certain institutional investors, including RA Capital Management, Vivo Capital, Venrock Healthcare Capital Partners, Farallon Capital Management, Acuta Capital, EcoR1 Capital LLC, Avidity Partners and Logos Capital at a price of $2.15 per share, a 12.6% premium to the February 27, 2020 closing price. The gross proceeds to Verastem Oncology were $100 million. After deducting the underwriting discounts and commissions and other estimated offering expenses, net proceeds to the Company were approximately $92.0 million.

Fourth Quarter 2019 Financial Results

Net product revenue for the three months ended December 31, 2019 (2019 Quarter) was $3.6 million, compared to $1.2 million for the three months ended December 31, 2018 (2018 Quarter), following the FDA's approval of COPIKTRA on September 24, 2018. COPIKTRA demand units for the 2019 Quarter increased 20% compared to the third quarter of 2019. There was no license and collaboration revenue for the 2019 or 2018 Quarter.

Total operating expenses for the 2019 Quarter were $36.9 million, compared to $35.5 million for the 2018 Quarter. Excluding non-recurring charges of $2.2 million related to the Convertible Notes Exchange, the total operating expenses for the 2019 Quarter were $34.7 million.

Research and development (R&D) expense for the 2019 Quarter was $12.5 million, compared to $8.8 million for the 2018 Quarter. The increase of $3.7 million, or 42.0%, was primarily related to higher contract research organization costs to support the development of the Phase 2 TEMPO study for Intermittent Dosing, pre-clinical collaborations, and personnel costs related to the October 2019 rightsizing of the organization. This is partially offset by a decrease in investigator fees and CMC costs related to the FDA Approval of COPIKTRA in 2018.

Selling, general and administrative expense for the 2019 Quarter was $23.7 million, compared to $26.2 million for the 2018 Quarter. The decrease of $2.5 million, or 9.5%, was primarily due to lower personnel and external consulting costs.

Net loss for the 2019 Quarter was $38.8 million, or $0.51 per share (diluted), compared to $11.3 million, or $0.37 per share (diluted), for the 2018 Quarter. The 2019 Quarter includes $1.3M of non-cash interest expense related to conversions of Convertible Senior Notes into shares of common stock.

For the 2019 Quarter, non-GAAP adjusted net loss was $30.3 million, or $0.40 per share (diluted), compared to non-GAAP adjusted net loss of $33.1 million, or $0.36 per share (diluted), for the 2018 Quarter. Please refer to the GAAP to Non-GAAP Reconciliation attached to this press release.

Full-Year 2019 Financial Results

Total revenue for the year ended December 31, 2019 (2019 Period) was $17.5 million. Net product revenue for the 2019 Period was $12.3 million, compared to $1.7 million for the year ended December 31, 2018 (2018 Period), following the FDA's approval of COPIKTRA on September 24, 2018. License and collaboration revenue for the 2019 Period was $5.1 million, compared to $25.0 million for the 2018 Period.

Total operating expenses for the 2019 Period were $149.8 million compared to $121.5 million for the 2018 Period.

R&D expense for the 2019 Period was $45.8 million, compared to $43.6 million for the 2018 Period. The increase of $2.2 million, or 5.0%, was primarily related to higher contract research organization and personnel costs to support the development of the Phase 2 TEMPO study for Intermittent Dosing and the Phase 2 PRIMO study for the treatment of PTCL.

Selling, general and administrative expense for the 2019 Period was $101.2 million, compared to $77.3 million for the 2018 Period. The increase of $23.9 million, or 30.9%, was primarily due to the hiring and staffing of the sales and commercial teams to support the launch of COPIKTRA.

Net loss for the 2019 Period was $149.2 million, or $2.00 per share (diluted), compared to $72.4 million, or $1.37 per share (diluted), for the 2018 Period.

For the 2019 Period, non-GAAP adjusted net loss was $126.0 million, or $1.69 per share (diluted), compared to non-GAAP adjusted net loss of $88.4 million, or $1.27 per share (diluted), for the 2018 Period. Please refer to the GAAP to Non-GAAP Reconciliation attached to this press release.

Verastem Oncology ended 2019 with cash, cash equivalents and short-term investments of $111.3 million.

Financial Guidance for Fiscal 2020

As a result of its new strategic direction, Verastem Oncology expects to reduce its operating expenses by approximately 40% for 2020 compared to 2019. Based on its current operating plans, Verastem Oncology expects its R&D and SG&A expenses for the full year 2020 to be in the range of $70 million to $85 million. In light of all these changes, the company is guiding that 2020 COPIKTRA revenues may be in the range of $12 million to $16 million. Verastem Oncology expects that its existing cash and cash equivalents, along with the revenue it expects to generate from COPIKTRA, will be sufficient to fund its planned operations into the fourth quarter of 2021.

Use of Non-GAAP Financial Measures

To supplement Verastem Oncology's condensed consolidated financial statements, which are prepared and presented in accordance with generally accepted accounting principles in the United States (GAAP), the Company uses the following non-GAAP financial measures in this press release: non-GAAP adjusted net loss and non-GAAP net loss per share. These non-GAAP financial measures exclude certain amounts or expenses from the corresponding financial measures determined in accordance with GAAP. Management believes this non-GAAP information is useful for investors, taken in conjunction with the Company's GAAP financial statements, because it provides greater transparency and period-over-period comparability with respect to the Company's operating performance and can enhance investors' ability to identify operating trends in the Company's business. Management uses these measures, among other factors, to assess and analyze operational results and trends and to make financial and operational decisions. Non-GAAP information is not

prepared under a comprehensive set of accounting rules and should only be used to supplement an understanding of the Company's operating results as reported under GAAP, not in isolation or as a substitute for, or superior to, financial information prepared and presented in accordance with GAAP. In addition, these non-GAAP financial measures are unlikely to be comparable with non-GAAP information provided by other companies. The determination of the amounts that are excluded from non-GAAP financial measures is a matter of management judgment and depends upon, among other factors, the nature of the underlying expense or income amounts. Reconciliations between these non-GAAP financial measures and the most comparable GAAP financial measures for the three and twelve months ended December 31, 2019 and 2018 are included in the tables accompanying this press release after the unaudited condensed consolidated financial statements.

About Verastem Oncology

Verastem Oncology (Nasdaq: VSTM) is a commercial biopharmaceutical company committed to the development and commercialization of new medicines to improve the lives of patients diagnosed with cancer. Our pipeline is focused on novel small molecule drugs that inhibit critical signaling pathways in cancer that promote cancer cell survival and tumor growth, including phosphoinositide 3-kinase (PI3K), focal adhesion kinase (FAK) and RAF/MEK inhibition.

Our first FDA approved product is available for the treatment of patients with certain types of indolent non-Hodgkin's lymphoma (iNHL).

For more information, please visit www.verastem.com.

Forward looking statements notice

This press release includes forward-looking statements about Verastem Oncology's strategy, future plans and prospects, including statements related to the opportunity to rapidly advance the development of clinical programs through Verastem Oncology's expanded development pipeline and strengthened balance sheet, the timing of top-line results for clinical trials, anticipated reductions in operating expenses from Verastem Oncology's strategic realignment, the timing of commencing a registration-directed trial for CH5126766 (VS-6766) and financial guidance estimates. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Each forward-looking statement is subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statement.

Each forward-looking statement is subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statement. Applicable risks and uncertainties include the risks and uncertainties, among other things, regarding: the success in the development and potential commercialization of our product candidates, including defactinib in combination with CH5126766 (VS-6766); the occurrence of adverse safety events and/or unexpected concerns that may arise from additional data or analysis or result in unmanageable safety profiles as compared to their levels of efficacy; our ability to obtain, maintain and enforce patent and other intellectual property protection for our 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 our product candidates; 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; that there may be competitive developments affecting our product candidates; that data may not be available when expected; that enrollment of clinical trials may take longer than expected; that our product candidates will experience manufacturing or supply interruptions or failures; 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 or Chugai Pharmaceutical Co., Ltd. will fail to fully perform under the CH5126766 (VS-6766) license agreement; that we may not have sufficient cash to fund our contemplated operations; 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 be unable to execute on our partnering strategies for defactinib in combination with CH5126766 (VS-6766); that we will not pursue or submit regulatory filings for our product candidates, 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 the Company’s Annual Report on Form 10-K for the year ended December 31, 2019, as filed with the Securities and Exchange Commission (SEC) on March 11, 2020 and in any subsequent filings with the SEC. The forward-looking statements contained in this press release reflect Verastem Oncology’s views as of the date hereof, and the Company does not assume and specifically disclaims any obligation to update any forward-looking statements whether as a result of new information, future events or otherwise, except as required by law.

Verastem Oncology Contacts:

Investors:
John Doyle
Vice President, Investor Relations & Finance
+1 781-469-1546
jdoyle@verastem.com

Media:
Lisa Buffington
Corporate Communications
+1 781-292-4205
lbuffington@verastem.com

Verastem, Inc.
Condensed Consolidated Balance Sheets
(in thousands)
(unaudited)

	December 31, 2019	December 31, 2018
Cash, cash equivalents, & investments	$ 75,506	$ 249,653
Accounts receivable, net	2,524	306
Inventory	3,096	327
Prepaid expenses and other current assets	3,835	2,973
Property and equipment, net	947	1,369
Intangible assets, net	20,008	21,577
Right-of-use asset, net	3,077	—
Restricted cash and other assets	36,053	1,031
Total assets	**$ 145,046**	**$ 277,236**
Current Liabilities	$ 29,890	$ 37,077
Long-term debt	35,067	19,506
Convertible senior notes	68,556	95,231
Lease Liability, long-term	3,489	—
Other liabilities	870	1,123
Stockholders' equity	7,174	124,299
Total liabilities and stockholders' equity	**$ 145,046**	**$ 277,236**

Verastem, Inc.
Condensed Consolidated Statements of Operations
(in thousands, except per share amounts)
(unaudited)

	Three months ended December 31,		Year ended December 31,	
	2019	**2018**	**2019**	**2018**
Revenue:				
Product revenue, net	$ 3,617	$ 1,210	$ 12,339	$ 1,718
License and collaboration revenue	—	—	5,117	25,000
Total revenue	3,617	1,210	17,456	26,718
Operating expenses:				
Cost of sales - product	332	116	1,238	165
Cost of sales - intangible amortization	393	392	1,569	423
Research and development	12,455	8,762	45,778	43,648
Selling, general and administrative	23,728	26,199	101,212	77,265
Total operating expenses	36,908	35,469	149,797	121,501
Loss from operations	(33,291)	(34,259)	(132,341)	(94,783)
Other (expense)/income	(641)	25,556	(641)	25,556
Interest income	611	1,306	4,381	2,603
Interest expense	(5,453)	(3,952)	(20,608)	(5,810)
Net Loss	$ (38,774)	$ (11,349)	$ (149,209)	$ (72,434)
Net loss per share—basic	$ (0.51)	$ (0.15)	$ (2.00)	$ (1.12)
Net loss per share—diluted	$ (0.51)	$ (0.37)	$ (2.00)	$ (1.37)
Weighted average common shares outstanding used in computing net loss per share—basic	76,331	73,766	74,578	64,962
Weighted average common shares outstanding used in computing net loss per share—diluted	76,331	91,061	74,578	69,321

Verastem, Inc.
Reconciliation of GAAP to Non-GAAP Financial Information
(in thousands, except per share amounts)
(unaudited)

	Three months ended December 31,		Year ended December 31,	
	2019	**2018**	**2019**	**2018**
Net Loss Reconciliation				
Net Loss (GAAP basis)	$ (38,774)	$ (11,349)	$ (149,209)	$ (72,434)
Adjust:				
Amortization of acquired intangible asset	393	392	1,569	423
Stock-based compensation expense	1,311	1,763	8,539	6,671
Non-cash interest, net	2,705	1,479	7,131	1,814
Severance and Other	1,232	218	3,200	710
Notes third party exchange costs	2,168	—	2,168	—
Change in fair value of interest make whole provision and conversion option for Notes	641	(25,556)	641	(25,556)
Adjusted Net Loss (non-GAAP basis)	$ (30,324)	$ (33,053)	$ (125,961)	$ (88,372)
Reconciliation of Net Loss Per Share				
Net Loss per share – diluted (GAAP Basis)	(0.51)	(0.37)	(2.00)	(1.37)
Adjust per diluted share				
Amortization of acquired intangible asset	0.01	0.00	0.02	0.01
Stock-based compensation expense	0.02	0.02	0.11	0.09
Non-cash interest, net	0.04	0.02	0.10	0.03
Severance and Other	0.01	0.00	0.04	0.01
Notes third party exchange costs	0.02	—	0.03	—
Change in fair value of interest make whole provision and conversion option for Notes	0.01	(0.03)	0.01	(0.04)
Adjusted Net Loss per share – diluted (non-GAAP Basis)	$ (0.40)	$ (0.36)	$ (1.69)	$ (1.27)
Weighted average common shares outstanding used in computing net loss per share—diluted	76,331	91,061	74,578	69,321