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: 001-38295

X4 PHARMACEUTICALS, INC.
(Exact name of registrant as specified in its charter)

Delaware	**27-3181608**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
955 Massachusetts Avenue, 4th Floor Cambridge, Massachusetts	**02139**
(Address of principal executive offices)	(Zip Code)

(857) 529-8300
(Registrant's telephone number, including area code)

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	XFOR	The Nasdaq Stock Market LLC

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 (of this chapter) during the preceding 12 months (of for such shorter period that the registrant was required to submit such files). 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 the 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 ☒

On June 30, 2019, the aggregate market value of the Registrant's voting common stock held by non-affiliates of the registrant was approximately $183,715,365 based upon the closing sale price on the Nasdaq Capital Market reported on June 28, 2019.

As of March 6, 2020, there were 16,134,495 shares of the registrant's common stock, $0.001 par value per share outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's definitive proxy statement, or the 2020 Proxy Statement, for its 2020 Annual Meeting of Stockholders, which the registrant intends to file pursuant to Regulation 14A with the Securities and Exchange Commission not later than 120 days after the registrant's fiscal year ended December 31, 2019, are incorporated by reference into Part III of this Annual Report on Form 10-K.

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EXPLANATORY NOTE

On March 13, 2019, X4 Pharmaceuticals, Inc. (formerly Arsanis, Inc.), or the Company, completed its business combination, or the Merger, in accordance with the terms of the Agreement and Plan of Merger, dated as of November 26, 2018, as amended on December 20, 2018 and March 8, 2019, or the Merger Agreement, by and among the Company, X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals, Inc.) and Artemis AC Corp., a Delaware corporation and a wholly-owned subsidiary of the Company, or the Merger Sub, pursuant to which, among other matters, Merger Sub merged with and into X4 Therapeutics, Inc., with X4 Therapeutics, Inc. continuing as a wholly-owned subsidiary of the Company and the surviving corporation of the merger. Following the Merger, on March 13, 2019, the Company effected a 1-for-6 reverse stock split of its common stock, or the Reverse Stock Split, and changed its name to “X4 Pharmaceuticals, Inc.” Following the completion of the Merger, the business conducted by X4 Pharmaceuticals Inc. became primarily the business conducted by X4 Therapeutics, Inc., a clinical-stage biopharmaceutical company focused on the research, development and commercialization of novel therapeutics for the treatment of rare diseases.

Unless otherwise noted, all references to common stock share and per share amounts in this Annual Report on Form 10-K have been retroactively adjusted to reflect the conversion of shares in the Merger based on an exchange ratio of 0.5702 and the Reverse Stock Split.

Unless the context requires otherwise, references in this Annual Report on Form 10-K to "X4", "we", "us" and "our" refer to X4 Pharmaceuticals, Inc. and its subsidiaries.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act, that relate to future events or to our future operations or financial performance. These statements may be identified by such forward-looking terminology as “may,” “should,” “expects,” “intends,” “plans,” “anticipates,” “believes,” “estimates,” “predicts,” “potential,” “continue” or the negative of these terms or other comparable terminology. Our forward-looking statements are based on a series of expectations, assumptions, estimates and projections about our company, are not guarantees of future results or performance and involve substantial risks and uncertainty. We may not actually achieve the plans, intentions or expectations disclosed in these forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in these forward-looking statements. These risks, uncertainties and other factors are described in greater detail under the caption “Risk Factors” contained in this Annual Report.

Our business and our forward-looking statements involve substantial known and unknown risks and uncertainties, including the risks and uncertainties inherent in our statements regarding:

- the progress, scope, cost, duration or results of our development activities, nonclinical studies and clinical trials of mavorixafor (X4P-001), X4P-002 and X4P-003 or any of our other product candidates or programs, such as the target indication(s) for development, the size, design, population, conduct, cost, objective or endpoints of any clinical trial, or the timing for initiation or completion of or availability of results from any clinical trial, including our current and planned trials for mavorixafor in Warts, Hypogammaglobulinemia, Infections, and Myelokathexis, or WHIM, syndrome, severe congenital neutropenia, or SCN, and Waldenström macroglobulinemia, or WM, for submission or approval of any regulatory filing or for meeting with regulatory authorities;
- the potential benefits that may be derived from any of our product candidates;
- the timing of and our ability to obtain and maintain regulatory approval of our existing product candidates or any product candidates that we may develop in the future, and any related restrictions, limitations, or warnings in the label of any approved product candidates;
- our plans to research, develop, manufacture and commercialize our product candidates;
- the timing of our regulatory filings for our product candidates, along with regulatory developments in the United States and other foreign countries;
- the size and growth potential of the markets for our product candidates, if approved, and the rate and degree of market acceptance of our product candidates, including reimbursement that may be received from payors;

- our commercialization, marketing and manufacturing capabilities and strategy;
- our ability to attract and retain qualified employees and key personnel;
- our competitive position;
- our expectations regarding our ability to obtain and maintain intellectual property protection;
- the success of competing therapies that are or may become available;
- our estimates and expectations regarding future operations, financial position, revenues, costs, expenses, uses of cash, capital requirements or our need for additional financing;
- our ability to raise additional capital; and
- our strategies, prospects, plans, expectations or objectives.

You should refer to "Risk Factors" for a discussion of important factors that may cause our actual results to differ materially from those expressed or implied by our forward-looking statements. As a result of these factors, we cannot assure you that the forward-looking statements in this Annual Report will prove to be accurate. Furthermore, if our forward-looking statements prove to be inaccurate, the inaccuracy may be material. In light of the significant uncertainties in these forward-looking statements, you should not regard these statements as a representation or warranty by us or any other person that we will achieve our objectives and plans in any specified time frame, or at all. We undertake no obligation to publicly update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law. You should, therefore, not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this Annual Report.

PART I

ITEM 1. BUSINESS

Overview

We are a clinical-stage biopharmaceutical company and a leader in the discovery and development of novel therapies for the treatment of diseases resulting from dysfunction of the CXCR4 pathway, with a focus on rare diseases with limited treatment options. We are led by an executive team with deep expertise in the science of the CXCR4 pathway and significant experience in the successful development and commercialization of therapeutics targeting rare diseases. Our lead therapeutic candidate, mavorixafor, is a first-in-class, oral small molecule inhibitor of the CXCR4 receptor, that we believe has the potential to treat a broad array of diseases, including primary immunodeficiencies, or PIs, and certain types of cancer.

Having established proof of concept and a favorable safety profile in a Phase 2 clinical trial, we initiated 4WHIM, a global Phase 3 clinical trial evaluating the safety and efficacy of mavorixafor for the treatment of patients with WHIM syndrome. WHIM syndrome is a rare, inherited, primary immunodeficiency disease caused by genetic "gain of function" mutations in the *CXCR4* receptor gene and named for its characteristic symptoms and some of its laboratory findings: **W**arts, **H**ypogammaglobulinemia, **I**nfections, and **M**yelokathexis. Mavorixafor was granted Breakthrough Therapy Designation by the U.S. Food and Drug Administration, or FDA, for the treatment of adult patients with WHIM syndrome in 2019. It was also granted orphan drug designation by the FDA in 2018 and by the European Commission in 2019 for the treatment of WHIM syndrome. We currently expect to report top-line results from 4WHIM in the second half of 2021.

We are also conducting Phase 1b clinical trials in two additional indications. The first clinical trial is investigating the safety and efficacy of mavorixafor in patients with Severe Congenital Neutropenia, or SCN, and severe idiopathic neutropenia, a group of rare blood disorders characterized by abnormally low levels of white blood cells, or neutrophils. The second clinical trial is a dose escalation, safety and preliminary efficacy trial in patients with Waldenström’s macroglobulinemia, or WM, a rare form of non-Hodgkin’s lymphoma in which abnormal white blood cells produce an excess of monoclonal immunoglobulin M, or IgM, which can result in symptoms of anemia, hyper viscosity, neuropathy, or other complications. Approximately 30-40% of patients with WM have a somatic mutation in the CXCR4 receptor suggesting a potential targeted approach to treatment with mavorixafor in combination with the standard BTK inhibitor (Ibrutinib) therapy. We are expecting the initial data results from each of these trials during the second half of 2020.

During 2019, we reported promising data from the Phase 2a portion of an open-label Phase 1/2 clinical trial studying mavorixafor for the treatment of patients with advanced clear cell renal cell carcinoma, or ccRCC, in combination with axitinib, an FDA-approved small molecule tyrosine kinase inhibitor. Future development and potential commercialization of mavorixafor in ccRCC in addition to other possible immuno-oncology indications will be pursued only as part of a potential strategic collaboration. .

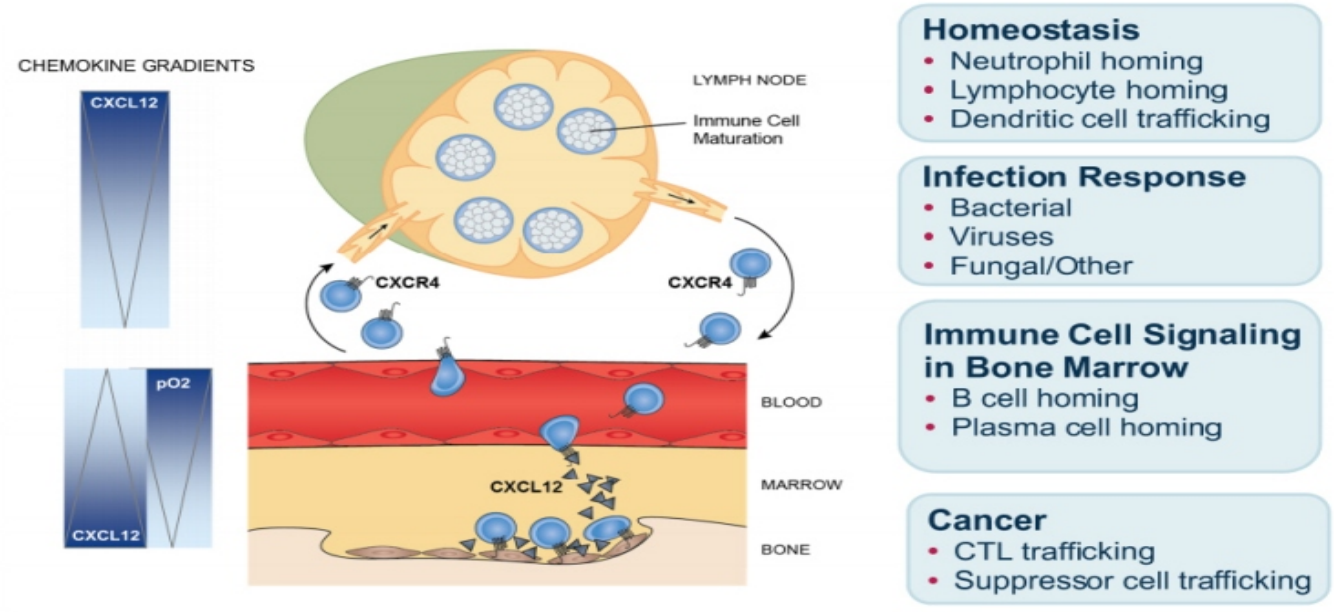
We are also advancing two early stage candidates towards the clinic: X4P-003, a second-generation CXCR4 antagonist designed to have an enhanced pharmacokinetic profile relative to mavorixafor, potentially enabling improved patient compliance and ease of use; and X4P-002, a CXCR4 antagonist designed to cross blood-brain barrier and provide appropriate therapeutic exposures to treat brain cancers.

We continue to leverage our insights into CXCR4 biology at our corporate headquarters located in Cambridge, Massachusetts and at our research facility in Vienna, Austria to discover and develop additional product candidates.

Our CXCR4 Platform

CXCR4, or C-X-C receptor type 4, is a G-coupled protein receptor, or GCPR, and its sole ligand is the chemokine CXCL12. Chemokines are signaling proteins that guide the migration of immune cells within the body by binding to receptors on the surface of target cells. When CXCR4 is stimulated by CXCL12, it plays a key role in enabling the trafficking of immune cells and effective immunosurveillance, or the process by which cells of the immune system look for and recognize foreign pathogens or precancerous and cancerous cells in the body (see Figure 1).

Figure 1: CXCL12/CXCR4 and Immune System Responses.



When the CXCL12/CXCR4 pathway is overstimulated, immune cells become immobilized, which can lead to immunosuppression and other complications from immune system dysfunction.

- In the case of primary immunodeficiencies, such as WHIM syndrome, overstimulation of the pathway is caused by mutations in the CXCR4 receptor, called “gain of function” mutations, that immobilize white blood cells in the bone marrow where they are produced and dramatically impact their ability to move into the blood and circulate throughout the body to perform immunosurveillance.
- In other diseases, including many types of cancer, the CXCL12/CXCR4 pathway has been found to broadly play a role in disrupted immune cell trafficking, particularly in the tumor microenvironment, or TME, where there often exists an abnormally high concentration of the ligand CXCL12. Evidence suggests that the pro-tumor signals between tumor cells and cancer-associated fibroblasts occur partly through chemokine signaling, including through the over-production of CXCL12, which results in immunosuppression within the tumor microenvironment.

We believe that the inhibition of the CXCL12/CXCR4 signaling pathway has the potential to improve immune cell trafficking and immunosurveillance and create therapeutic benefit across a wide variety of diseases.

We also believe that our approach to inhibiting the CXCR4 receptor has been validated by the FDA-approved product plerixafor for injection (marketed as Mozobil). Plerixafor is a CXCR4 antagonist that has been shown to induce white blood cell mobilization and is used as an injectable therapy for short-term treatment in preparation for stem-cell transplants. In a published investigator-sponsored pilot study of WHIM patients, twice-daily injections of low-dose plerixafor demonstrated increased white blood cell counts and reduced infections and wart lesions. Plerixafor is not approved for the treatment of WHIM syndrome nor are we aware of any plans to develop and commercialize it as a treatment for patients with WHIM syndrome or any other immunodeficiencies.

Our leadership team has considerable experience in the research, development, and commercialization of therapies to treat rare diseases, including therapies that target chemokine pathways and the CXCR4 pathway specifically. Paula Ragan, Ph.D., our Chief Executive Officer, led the licensing of the CXCR4 antagonist portfolio from Sanofi-Genzyme and coordinated all phases of the transfer of the knowledge and know-how needed to launch our company. Our co-founder and our current Senior Vice President of Research and Development, Renato Skerlj, Ph.D., is very experienced in the discovery and development of small molecule drugs targeting rare diseases, cancer, infection, and neurodegenerative disease, and is an inventor of plerixafor. Two members of our Board of Directors also have deep roots in our differentiated chemokine approach, including Gary J. Bridger, Ph.D., who was responsible for the discovery and development of plerixafor as a co-founder and Chief Scientific Officer of AnorMED Inc., until

the company's acquisition by Genzyme in 2006, and Michael S. Wyzga, Chairman of our Board of Directors, who was the Chief Financial Officer of Genzyme during the approval, global launch, and subsequent commercialization of plerixafor. We believe that the experience of our leadership team provides our company with unique insights into product development and commercialization processes and the identification of opportunities involving CXCR4 biology.

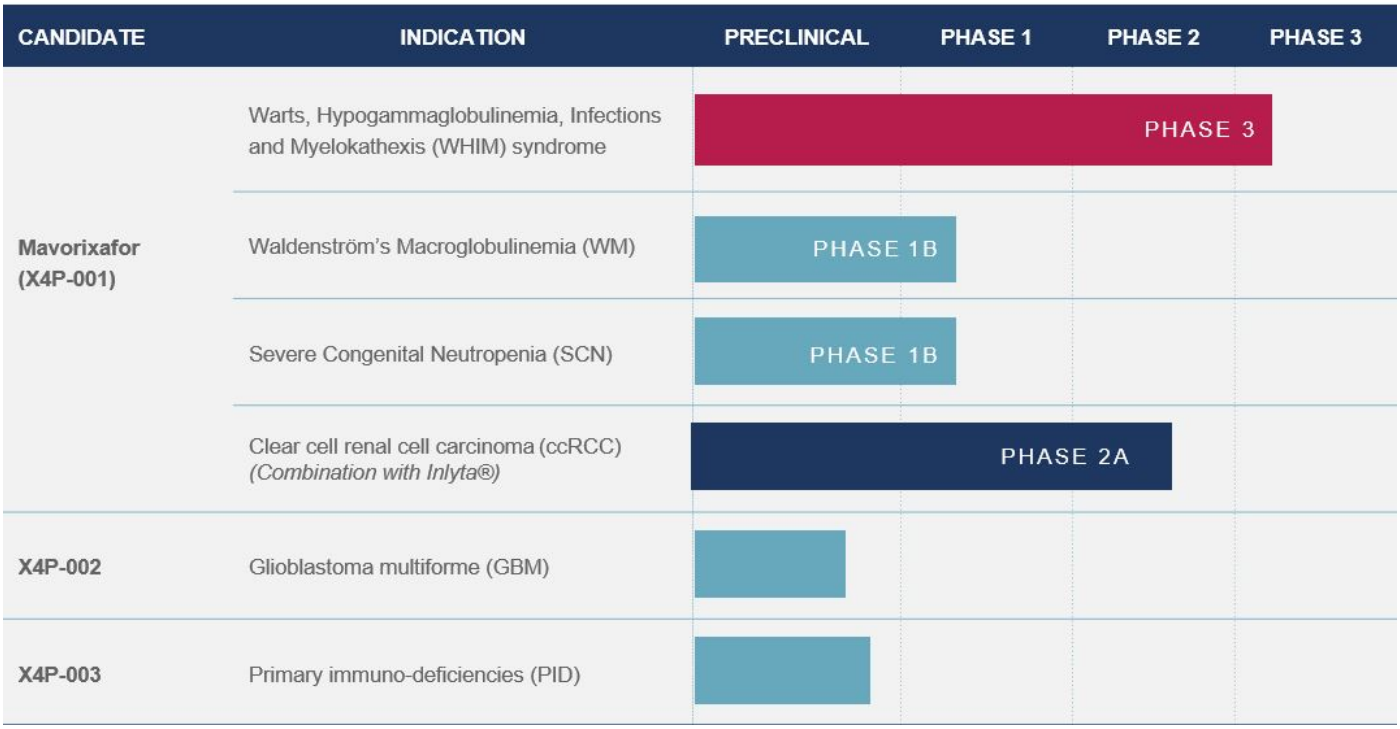
Our Strategy

Our goal is to discover, develop, and commercialize novel therapeutics that address diseases resulting from dysfunction of the CXCR4 pathway by:

- **Completing the global Phase 3 clinical trial to enable the approval of mavorixafor for the treatment of patients with WHIM syndrome**. To date, we have completed a Phase 2 clinical trial of mavorixafor in patients with WHIM syndrome, achieving clinical proof-of-concept, observing a clinically meaningful increase in neutrophil and lymphocyte counts, and demonstrating a favorable tolerability profile. We have initiated a Phase 3 pivotal clinical trial, the 4WHIM trial, and are currently enrolling patients, with top-line data expected in the second half of 2021.
- **Driving community awareness of WHIM syndrome and building patient registries**. We are building our efforts to increase awareness of underserved serious rare diseases, including WHIM syndrome, among patients, physicians, and their support systems. In addition to sponsored market research and outreach, we have partnered with key patient foundations and registries, including the Jeffrey Modell Foundation, University of Washington, Immune Deficiency Foundation, and Hopitaux Universitaires Est Parisien (Trousseau La Roche-Guyon). In addition, we have deployed a field force of Medical Science Liaisons, or MSLs, in the United States to further drive education and awareness of WHIM syndrome. We plan to leverage our relationship with our partner organizations and patient registries along with our MSL team in the United States to increase patient and physician awareness of our ongoing trials, supporting study enrollment and completion.
- **Advancing mavorixafor in additional indications.** To maximize the potential of mavorixafor, we have initiated two Phase 1b clinical trials – one trial investigating the safety and efficacy of mavorixafor in patients with SCN and another trial investigating the safety, appropriate dose and preliminary efficacy of mavorixafor in patients with Waldenström's macroglobulinemia. We are expecting the initial data results from each of these studies during the second half of 2020.
- **Advancing earlier-stage product candidates and leveraging insights into CXCR4 biology to further expand our pipeline.** We have advanced a late lead optimization program into candidate drug nomination: X4P-003, a second-generation CXCR4 antagonist designed to have an enhanced pharmacokinetic profile relative to mavorixafor, potentially enabling improved patient compliance and ease of use. A second program is in lead optimization: X4P-002, a CXCR4 antagonist designed to cross the blood-brain barrier and provide appropriate therapeutic exposures to treat brain cancers. In addition, we are also leveraging our insights into CXCR4 biology and our research capabilities to identify other targets and develop additional product candidates.
- **Independently commercializing our product candidates in certain indications and geographies where we believe we can maximize value.** Given the potential of our product candidates to treat a wide variety of diseases, we believe that it will be important to maintain our leadership role with respect to our development and commercialization efforts. We plan to independently develop therapeutic candidates in indications, including rare diseases, where we believe there is a well-defined clinical and regulatory approval pathway and ones that we believe we could successfully commercialize, if approved.
- **Pursuing additional strategic collaborations for mavorixafor in immuno-oncology indications**. Currently, we have an ongoing strategic collaboration under which we have licensed mavorixafor rights in China to Abbisko, a privately-held immuno-oncology research and development company. Additionally, we intend to pursue strategic collaborations for future development and potential commercialization of mavorixafor in solid tumor immuno-oncology indications in other global regions in order to maximize the value of that asset while we maintain our focus on developing mavorixafor for the treatment of rare diseases.

Our Programs

We are advancing a pipeline of clinical and pre-clinical oral, small molecule candidates that target diseases resulting from the dysfunction of the CXCR4 pathway, including primary immunodeficiencies and certain types of cancer.



Mavorixafor

Our lead product candidate is mavorixafor, a first-in-class, oral, selective small molecule CXCR4 antagonist that allosterically, or non-competitively, inhibits receptor binding by CXCL12, the cognate ligand of CXCR4. Mavorixafor is designed to correct the abnormal signaling caused by the dysfunction of the receptor/ligand interaction and enable mobilization and trafficking of immune cells, increasing levels of circulating white blood cells, including neutrophils, to improve immune system function.

To date, more than 190 patients in clinical trials have been dosed with mavorixafor, which has demonstrated a favorable tolerability profile. In these trials, we have observed drug exposure in patients, a 23-hour half-life, and the bioavailability of mavorixafor to support once-daily oral dosing, which we believe will provide convenient dosing and better patient compliance for chronic or life-long use, if approved. The manufacturing process for mavorixafor utilizes well established small molecule chemistry, enabling a potential commercial product that can be supported by specialty pharmacy distribution.

Mavorixafor in WHIM Syndrome

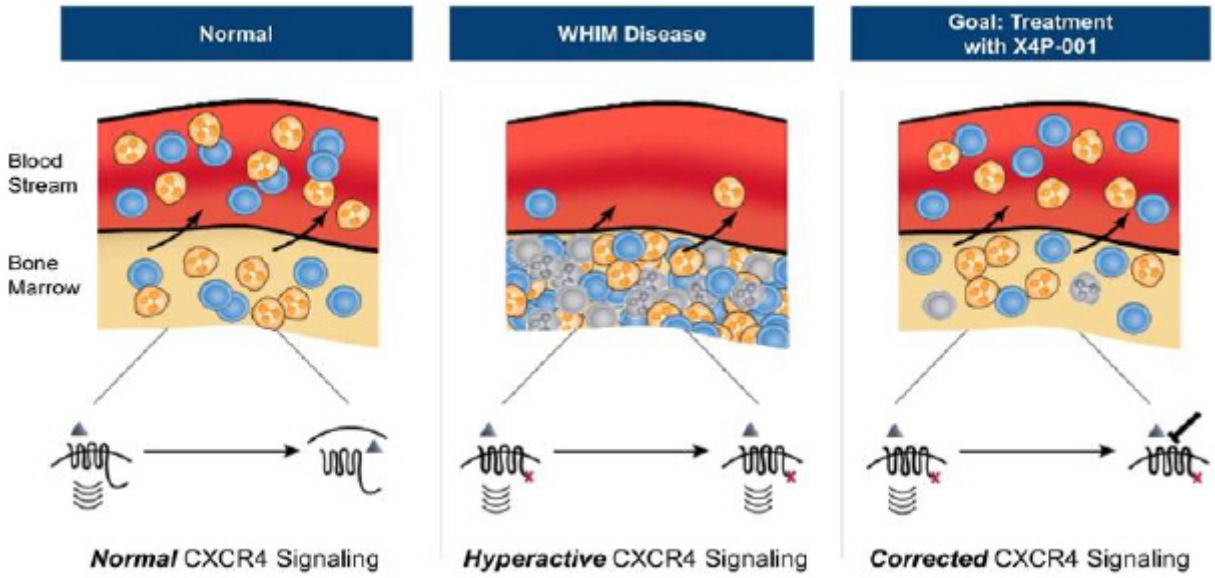
Primary Immunodeficiencies, or PIs, are a group of more than 300 rare, chronic disorders in which flaws in the immune system cause increased susceptibility to infections and, in some cases, increased risk of cancers. Within this broad disease classification, WHIM syndrome is one of a number of PIs that are caused by the improper trafficking of immune cells. WHIM syndrome is a rare genetic disease that results from "gain of function" mutations in the single gene that encodes for the CXCR4 receptor. The first of these mutations were identified in 2003. Since then, a total of 11 different CXCR4 mutations have been identified as causing WHIM syndrome. All but one of these mutations cause premature truncations in the protein, causing the receptor to signal for longer than normal, which results in the retention of white blood cells in the bone marrow where they are produced, and leads to the chronic peripheral neutropenia and lymphopenia that is the observed clinical hallmark of WHIM syndrome.

The incidence and prevalence of WHIM syndrome are not well established. We believe that this is due to the relatively recent understanding of the genetics underlying WHIM syndrome, lack of universal or accessible genetic testing, and limited medical education and awareness of the disease, which is in part driven by the lack of available and disease-modifying treatments. The National Organization for Rare Diseases, or NORD, has reported the incidence of WHIM syndrome to be less than one in 1,000,000 based on a single small registry of eight patients in France. Based on a preliminary independent market research study that we sponsored in the United States, which was conducted by a third party research firm, we believe that the prevalence of WHIM syndrome worldwide is significantly higher than the incidence statistics implied by the France-based registry. The study solicited input from community-based physicians of different specialties, including physicians focused on non-malignant hematology, immunology, dermatology, pulmonology and infectious diseases, who are known to manage and/or treat patients

with WHIM syndrome. The 212 physicians across these specialties in the United States identified to participate in this study reported over 1,700 patients have genetically confirmed or are highly suspected to have WHIM syndrome in the United States alone. The results of this initial study support our estimate of more than 1,000 genetically confirmed WHIM patients in the United States.

Figure 2 illustrates the mutations in the CXCR4 receptor leading to abnormal signaling and retention of white blood cells in the bone marrow that occurs in WHIM patients. Figure 2 also depicts our approach to blocking this abnormal signaling using our CXCR4 antagonist candidate, mavorixafor, which is designed to enable white blood cells to release into the bloodstream, restoring normal immune function. In healthy individuals, the CXCR4 receptor is typically internalized into the cell after CXCL12 binds to it, enabling the receptor to be appropriately "recycled" and the signaling to be diminished. In WHIM patients, however, a mutation truncates the intracellular portion of the CXCR4 receptor as shown by the red "x" below, which prevents the post-binding internalization ("normal recycling") of the receptor. As a result, the CXCR4 receptor is maintained on the surface of the cell and is exposed to the ligand, which creates a perpetual "on" signaling and immobilizes the cell. Mavorixafor binds to the mutated CXCR4 receptor in a manner that blocks the receptor from being stimulated by CXCL12 regardless of the presence of the ligand, and results in increased mobilization and trafficking of white blood cells from the bone marrow.

Figure 2. WHIM Syndrome: Genetic Mutations in CXCR4 Create Abnormal Trafficking of White Blood Cells



In addition to life-long neutropenia and lymphopenia, WHIM patients can clinically present with other manifestations of the disease, such as warts related to infection with the Human Papilloma Virus, or HPV. Based on publications from the French Severe Congenital Neutropenia Registry and the broader literature, it is estimated that between 30% and 40% of WHIM patients develop HPV-associated cancers as they age. Patients may also demonstrate low immunoglobulin, or IG, levels, also known as hypogammaglobulinemia and pathology assessments of bone marrow samples (aspirates) of patients with WHIM syndrome show a "hyper-dense" population of pre-apoptotic immune cells in the bone marrow, a condition known as myelokathexis. These conditions reduce the body's ability to achieve a healthy immune response.

For a diagnosis of WHIM syndrome, all four classic characteristics of warts, hypogammaglobulinemia, infections and myelokathexis, do not need to be present, which complicates the diagnosis of these patients. Genetic testing is used to definitively diagnose WHIM syndrome by confirming the presence of an autosomal dominant mutation in the CXCR4 receptor where only one mutated gene need be affected to cause the disorder. The diagnosis of WHIM syndrome may occur at any age: about one-half of reported patients are diagnosed as adults, mostly between 18 and 40 years of age, with the other half diagnosed primarily before or at the age of 18 years.

Clinical Development of Mavorixafor in WHIM Syndrome

In January 2017, we initiated a Phase 2 clinical trial of mavorixafor for the treatment of patients with WHIM syndrome. This trial was an open-label, dose-escalation trial in eight WHIM patients conducted at two sites in the United States and Australia pursuant to an Investigational New Drug, or IND, application that we submitted to the FDA in June 2016. The primary objective of the Phase 2 clinical trial was to determine the safety and tolerability of mavorixafor and to determine the dose of mavorixafor for exploration in a Phase 3 pivotal clinical trial. The secondary objective of the Phase 2 trial was to evaluate the potential efficacy of mavorixafor in patients with WHIM syndrome by measuring biomarkers, specifically absolute neutrophil and lymphocyte counts, or ANCs and ALCs, over 24-hour dosing cycles. The frequency of infections, antibiotic use, hospitalizations, severity of warts lesions, and vaccine titer levels, among other metrics, were also examined. To be included in the trial, patients must have had a confirmed genetic diagnosis of WHIM syndrome, be at least 18 years of age and have a neutrophil count equal to or less than 400/µL or a lymphocyte count equal to or less than 650/µL. Patients who had been infected with the human immunodeficiency virus, or HIV, were excluded from the trial, as were patients with recent exposure to plerixafor.

In the trial, patients received escalating doses of mavorixafor starting at 50 mg once daily to up to 400 mg once daily. Patients received starting doses higher than 50 mg once daily as the trial progressed based on the safety and biomarker response data of earlier patients enrolled in the trial. Patients were dose-escalated from their starting dose based on an in-hospital 24-hour measurement of ANCs and ALCs above or below the pre-defined target thresholds of 600/µL and 1,000/µL, respectively. Results were as follows:

- Mavorixafor was not associated with any treatment-related serious adverse events and was observed to be well tolerated in daily doses of up to 400 mg for durations of up to 400 days. Treatment-related adverse events reported were dry mouth (n=2), nausea (n=4), dry eye (n=1), nasal dryness (n=2), dyspepsia (n=1), conjunctivitis (n=1) and rash (n=1). All adverse events were Grade 1 (mild).
- ANCs were increased in total numbers (cells//µL) as well as duration of increase (hours) over the course of the intra-day assessment. The increase in ANCs vs. pre-dosing levels was observed in all (n=7) evaluable patients in the trial. In five of seven patients (71%), ANCs were consistently elevated above the pre-defined target threshold of 600/µL at peak as well as throughout the 24 hour assessment period.
- Similarly, ALCs were increased in total numbers (cells/ µL) as well as duration of increase with the treatment of mavorixafor. The increase in ALCs were observed in all (n=7) evaluable patients with six of seven patients (86%) exceeding the pre-defined target threshold of 1,000/µL for ALCs (the lower limit of normal) throughout the 24-hour assessment period. These thresholds of 600/µL for ANCs and 1,000/µL for ALCs correspond to the National Cancer Institute's adverse event grading system, which considers ANCs below 500/µL to be severe or life threatening and ALCs of 1,000/µL within the range of healthy individuals.
- Patients experienced improved infection rates, as reported by patients and the trial investigators.
- Significant and visible reductions in wart lesions were also reported in a patient with a history of untreatable severe wart lesions.

We completed the dose-titration portion of the Phase 2 trial in March 2018 and, based on the reported results, the Data Review Committee, or DRC, recommended the Phase 3 dose of 400 mg administered once daily based on these results. Following completion of the dose-titration portion of the Phase 2 trial, patients were allowed to continue on study drug in a Phase 2 open-label extension trial. As of February 2020, three patients continue to receive mavorixafor in the open-label extension trial.

In June 2019, we initiated 4WHIM, a global, Phase 3 pivotal clinical trial investigating the safety and efficacy of mavorixafor in genetically confirmed WHIM patients. 4WHIM trial is a 52-week, randomized, double-blind, placebo-controlled, multicenter study designed to evaluate the safety and efficacy of mavorixafor in genetically confirmed WHIM patients. The trial is designed to enroll up to 28 subjects in approximately 20 countries, followed by an open-label extension trial. The primary efficacy endpoint for the trial will compare the time above threshold, or TAT, defined as the number of hours that circulating neutrophils are above a clinically meaningful threshold (500 cells/µL), in response to mavorixafor versus placebo as measured during a 24-hour period. Patients will be assessed for his or her neutrophil TAT approximately once every three months (4 times in total) over the course of the 52 week treatment period. Secondary endpoints include infection rates, wart burden, and assessments of immune system function and quality of life. Upon completion of the treatment period, patients will be eligible to participate in an open-label extension, or OLE, and receive mavorixafor until the drug is commercially available.

We currently expect to report top-line data from this Phase 3 trial in the second half of 2021. If the Phase 3 pivotal clinical trial is successful, we believe that this trial will be the only registration trial required to submit a New Drug Application, or NDA, to the FDA seeking regulatory approval in the United States. Mavorixafor was granted Breakthrough Therapy Designation by the FDA

for the treatment of adult patients with WHIM syndrome in 2019, and was granted orphan drug designation by the FDA in 2018 and by the European Commission in 2019 for the treatment of WHIM syndrome.

Limited Current Treatment Landscape for WHIM Syndrome

Currently, there are no approved targeted therapies for the treatment of WHIM syndrome and care is limited to the treatment of the syndrome's different symptoms. The care of WHIM patients is mainly focused on the prevention and management of infections. None of these treatments, however, have been clinically proven to be effective for treating WHIM syndrome nor do they address the underlying cause of this multi-faceted disease, the genetic defect of the CXCR4 receptor. Current symptoms and their limitations are as follows:

- ***Warts:*** The presence of warts in WHIM syndrome is driven by an underlying HPV infection. Standard treatments, such as topical therapies (for example, imiquimod and salicylic acid), cryotherapy and laser therapy, as well as more aggressive approaches, such as cauterization or surgical removal, have been ineffective in providing durable treatment of warts associated with chronic HPV infections. As WHIM patients generally have limited response to vaccines, the HPV vaccine appears to have limited effectiveness. The number, size and severity of visible warts in WHIM patients can have a significant negative impact on the patient's quality of life and result in social anxiety issues. Left untreated, chronic HPV-infections are also known to increase the risk of cancer. Patients with WHIM syndrome are reported to have more frequent occurrences of difficult to treat HPV-associated cancers, such as head and neck and anogenital cancers.

- ***Hypogammaglobulinemia:*** Intravenous or subcutaneous Ig administration, referred to as IVIg or SCIg, respectively, can be administered to patients with low Ig levels. In WHIM patients, the administration of Ig therapies raises Ig levels, but has shown no impact on circulating leukocytes and limited or no impact on immune responses. Ig treatment of patients with WHIM syndrome is based on empirical and anecdotal evidence, and there are no clinical data demonstrating the efficacy of Ig treatment for WHIM syndrome. Ig treatment also does not treat or protect against HPV-associated symptoms and diseases, such as warts and certain cancers. Furthermore, Ig administration is costly and time consuming.

- ***Infections:*** Bacterial infections are managed with antibiotics. Acute infections usually resolve, although we are aware of reports from clinicians citing death due to pneumonia or sepsis in young WHIM patients. Importantly, even with antibiotic use, infections recur more frequently and persist longer in patients with WHIM syndrome. Further, the toll of multiple, chronic infections in WHIM patients has been known to lead to devastating irreversible pathologies such as hearing loss due to chronic ear infections and bronchiectasis. Patients are sometimes given a granulocyte-colony stimulating factor, or G-CSF, to increase neutrophil counts, but G-CSF has demonstrated little, if any, impact on lymphopenia or the incidence of infections in WHIM patients. In a small registry of eight WHIM patients in France, three of the four patients who received G-CSF continued to have persistent, repeated infections. Side effects of G-CSF have been reported to include disabling bone pain, which can be more severe in certain age groups. Additional, less common, treatment-limiting complications of chronic G-CSF administration include myelofibrosis and leukemia.

- ***Myelokathexis:*** G-CSF is sometimes used to treat the myelokathexis characteristic of WHIM syndrome to try to increase the number of neutrophils outside of the bone marrow, but G-CSF has no effect on lymphocytes and other types of white blood cells. Side effects of G-CSF can include disabling bone pain, myelofibrosis and leukemia.

While the costs of managing the chronic impact of WHIM syndrome are unknown, the per-patient cost of treating PIs that are similar to WHIM syndrome based on drug costs alone exceeds $100,000 per year in the United States utilizing similar therapies, such as antibiotics, IVIg, SCIg and/or G-CSF, despite the limited effectiveness of these treatments. Beyond these estimated direct costs, other costs associated with direct and indirect management of the disease, such as repeated immunization, physician visits, or hospitalizations, have not been quantified but are likely to be significant. We believe that there is a significant need for a treatment targeting the underlying excessive signaling caused by mutations to the CXCR4 receptor, which is the established cause of WHIM syndrome.

Mavorixafor in SCN

We believe that mavorixafor may be used to treat a number of additional PIs beyond WHIM syndrome. Particularly, we believe that mavorixafor can potentially treat patients with severe congenital neutropenia, or SCN. Like WHIM syndrome, SCN is a rare blood disorder similarly characterized by increased risks of infections and cancer due to abnormally low levels of neutrophils in the body. Additionally, some sub-types of SCN have mechanisms that overlap with signaling of the CXCL12/CXCR4 pathway. Affecting an estimated 2,000 to 3,000 people in the United States and Europe, patients with SCN are prone to recurrent, often life-threatening infections beginning in their first months of life.

G-CSF is the current standard of care for SCN and is used to stimulate the bone marrow to produce neutrophils. Side effects of G-CSF include disabling bone pain, which can be more severe in certain age groups. Additional, less common, treatment-limiting complications of chronic G-CSF administration include myelofibrosis and leukemia. In SCN cases that are unresponsive to G-CSF, or if leukemia has developed, bone marrow transplants have been made with varying degrees of success. Bone marrow transplants bring additional risks into the management of the disorder.

In November 2019, we initiated a Phase 1b clinical trial, a 14-day, proof-of-concept trial designed to assess the safety and tolerability of daily, oral mavorixafor in participants with SCN and other selected congenital neutropenia disorders. In addition, the trial will evaluate the neutrophil response in this patient population as an independent agent or in combination with G-CSF. The trial is expected to enroll up to 45 patients in total, with initial data results anticipated in the second half of 2020.

Mavorixafor in Waldenström's macroglobulinemia
We believe that mavorixafor may be used to treat certain blood cancers, including Waldenström's macroglobulinemia, or WM. WM, is a rare form of non-Hodgkin’s lymphoma and B-cell lymphoproliferative disorder most often caused by mutations in a gene for MYD88. The WM landscape has recently been revolutionized by whole-genome sequencing that has identified genetic mutations in the disease, including secondary mutations in CXCR4. Approximately 30-40% of all WM patients have been shown to have second mutation, a somatic WHIM-like mutation in the CXCR4 gene in the cancer cells that define this rare form of lymphoma. Mutational status influences both clinical presentation and prognosis as well as provides potential implications for therapeutic options. Patients with the CXCR4 mutations have higher serum IgM levels and greater incidence of symptomatic hyperviscosity. These patients also have a worse prognosis.

In WM, somatic mutations of CXCR4 have been found to be associated with activating and pro-survival signaling of tumor cells, as well as the possible acquisition of resistance to several drugs, including anti-CD20 monoclonal antibody, and BTK inhibitors, such as ibrutinib, the current standard of care. For example, WM patients who have been treated with ibrutinib and patients with WHIM syndrome-like mutations have generally not responded as well to treatment as compared to patients without the somatic WHIM syndrome mutation. Seminal work from Dr. Steven P. Treon M.D. Ph.D., of Harvard Medical School, has demonstrated that patients with the double mutation (MYD88/CXCR4 WHIM) have a lower rate of Very Good Partial Response, or VGPR, and Minor Response, or MR, as well as longer time to MR (31% vs. 7% VGPR, 94% vs. 71% for MR and 1 month to MR in the single mutant vs. 7.3 months in the double mutation). This is true in treatment naive patients with similar worse outcomes in the double mutation population shown by both Dr. Treon and Dr. Christian Buske M.D. in previously treated patients.

In December 2019, we initiated a Phase 1b multi-center, open-label, dose-escalation, clinical trial designed to assess the safety and tolerability of mavorixafor in combination with ibrutinib in patients with WM who have acquired a “gain of function” mutation in CXCR4 in addition to the MYD88 mutation. In addition, the trial is designed to measure changes from baseline in serum immunoglobulin M and hemoglobin, both key biomarkers of clinical response in WM patients. The clinical trial is expected to enroll between 12-18 patients, with initial data results expected in the second half of 2020.

This trial is being conducted as part of a collaboration with The Leukemia & Lymphoma Society, or LLS, to accelerate the development of mavorixafor for the treatment of WM. Mavorixafor was selected for LLS’s Therapy Acceleration Program®, or TAP, a strategic initiative where LLS builds business alliances and collaborations with biotechnology companies and academic researchers to speed the development of new therapies for blood cancers.

Mavorixafor in Immuno-Oncology Indications
In solid tumors, the tumor micro-environment, or TME, consists of the tumor cells and cancer associated fibroblasts, or CAFs, each of which overproduce growth factors and chemokines to support immune-suppression and malignant cell proliferation and growth. Evidence suggests that the pro-tumor signals between tumor cells and CAFs occur, in part, through chemokine signaling, including through the over-production of CXCL12. The CXCL12/CXCR4 pathway has been shown to be overstimulated in more than 20 solid and blood-derived tumor types. Excessive stimulation of CXCR4 due to high concentrations of CXCL12 influences trafficking immune cells, including myeloid-derived suppressor cells, or MDSCs, CD4+ regulatory T-cells, or Tregs, CD8+ T-cells, and mature dendritic cells. We believe that blocking CXCR4 overstimulation can lead to improved immune cell trafficking and increase the absolute number of CD8+ T-cells, thereby also increasing the ratio of CD8+ T-cells to Tregs in the TME.

Our Phase 1b Clinical Trial in Combination with Nivolumab in ccRCC

We have completed a Phase 1b trial of mavorixafor for the treatment of patients with ccRCC in combination with the approved immuno-oncology therapy, nivolumab, a PD-1 checkpoint inhibitor. The primary objective of the trial was to evaluate the safety and tolerability of mavorixafor in combination with nivolumab. The trial enrolled patients who have not responded to nivolumab, but who were maintained on nivolumab while mavorixafor was added to their treatment regimen. In addition to safety and tolerability, the trial evaluated early signs of biological activity using biomarkers, and clinical activity as measured by ORR.

Enrolled patients received 400 mg of mavorixafor once daily and continued to receive standard bi-weekly nivolumab therapy. Median duration of treatment with the combination was 3.7 months (range one to 15 months). We observed that five of nine patients had clinical improvements in tumor shrinkage with the addition of mavorixafor.

In the trial, we observed that mavorixafor in combination with nivolumab had an acceptable tolerability profile in ccRCC patients. The most frequent drug related adverse events were diarrhea, nasal congestion, ALT/AST increase, dry eye and fatigue. No Grade 4 or 5 adverse events occurred. All Grade 3 serious adverse events related to the combination treatment were reported to be manageable with appropriate intervention. Two patients experienced serious adverse events deemed unrelated to treatment: one had mucosal inflammation and rash maculo-papular and another had an ALT/AST increase and autoimmune hepatitis.

In addition, in the trial, combination therapy with mavorixafor and nivolumab exhibited anti-tumor activity in some patients with advanced ccRCC who were previously unresponsive to nivolumab monotherapy. Four patients who had progressed on prior nivolumab monotherapy were observed to have a best response of stable disease with the additional mavorixafor to nivolumab treatment. Of the five patients who were stable on prior nivolumab monotherapy, one had a partial response with combination therapy of mavorixafor and nivolumab.

Our Phase 1b Clinical Biomarker Trial in Advanced Melanoma

We have completed a Phase 1b biomarker clinical trial in 16 patients with Stage III and IV melanoma. This multi-center trial evaluated the safety and tolerability of mavorixafor alone and in combination with the immuno-oncology therapy pembrolizumab, an approved PD-1 checkpoint inhibitor. The trial evaluated the immune profile of participants' tumor biopsies and blood to assess changes of key immune cell profiles and inflammatory response markers. Nine patients had both baseline (pre-dose) and post-mavorixafor treatment-evaluable biopsies and were considered as evaluable patients to be included in the analysis. Results from the tumor biopsies taken from melanoma patients, before and after receiving single agent mavorixafor treatment for three weeks, were analyzed. Analyses showed three weeks of single agent mavorixafor monotherapy was associated with tumor immunity. Enhanced immunity was indicated by:

- increased proliferating CD8+ cells, indicative of cytotoxic T-cell activation;
- increased IFN-gamma gene expression signature score, suggesting enhanced antigen priming and activation;
- increased Tumor Inflammation Signature, or TIS, indicative of increased inflammation status in the TME;
- increased CD8+ T-cell density at the tumor interface, with the total density of CD8+ cells inside the tumor boundary area increased four-fold compared with baseline;
- increased numbers of cells expressing CD3 antigens, a pan T-cell marker, within tumor borders, and decreased expression of VISTA, a checkpoint molecule that inhibits T-cell activation and proliferation; and/or
- increases in multiple chemoattractant factors in serum, consistent with increased trafficking of immune cells post CXCR4 inhibition.

After single agent mavorixafor treatment, patients received mavorixafor in combination with pembrolizumab for an additional six weeks. Continued signs of positive immune cell changes in the TME were seen with combination treatment. Treatment of additional patients in the trial showed that mavorixafor as a single agent, and in combination with pembrolizumab, continued to be well tolerated. Mavorixafor was well tolerated as monotherapy and in combination with pembrolizumab. Treatment-related adverse events were diarrhea, fatigue, rash macro-papular and dry eye. No Grade > 3 adverse events were observed during the monotherapy period and there were no Grade 4 or 5 adverse events at any time during the period of the trial.

Our Phase 1/2 Clinical Trial in Combination with Axitinib in ccRCC

We have also completed an open-label, Phase 1/2 clinical trial of mavorixafor for the treatment of patients with advanced ccRCC who had received at least one prior line of therapy across multiple sites in the United States and South Korea.

This Phase 1/2, multi-center, open-label trial of mavorixafor in combination with the approved tyrosine kinase inhibitor axitinib included 65 patients with histologically confirmed advanced ccRCC, all of whom received at least one prior systemic therapy.

The safety analyses included 65 patients from Phases 1/2 of the trial who were treated with 400 mg mavorixafor (200 mg twice daily or 400 mg once daily) + 5 mg axitinib twice daily. Treatment responses were assessed using Response Evaluation Criteria in Solid Tumor, or RECIST v1.1 (a validated set of criteria to assess changes in tumor burden), every eight weeks from day one for 80 weeks, and then every 12 weeks thereafter, by blinded, independent central review. Treatment-related serious adverse events were diarrhea, hyperkalemia and hypertension (n=2, or 3%) and blood creatinine increased, dehydration, fatigue, hepatic enzyme increase, nausea, sepsis, trachea-oesophageal fistula, and vomiting (n=1 each, or 1.5%).

In the Phase 2a portion of the trial, combination therapy with mavorixafor and axitinib was generally well tolerated with a manageable safety profile and demonstrated clinical improvement with encouraging median progression free survival, or mPFS, in a heavily pre-treated advanced ccRCC patient population. Of the 65 patients in the trial, 49 patients (or 75%) received mavorixafor + axitinib as a third- to ninth-line therapy, having received between two and eight prior therapies with a TKI, immuno-oncology, or IO, agent, or other systemic therapy. Fifty-seven of the 65 patients in the trial (or 88%) had an intermediate or poor prognosis.

Overall mPFS across clinically evaluable patients receiving mavorixafor + axitinib (n=62) was 7.4 months. Predefined subpopulations examined patients with immediate prior TKI and IO treatment. Patients treated in the subgroup with immediate prior TKI therapy (n=34) demonstrated an objective response rate, or ORR, of 18% and an increased mPFS of 7.4 months. This is a greater than 50% improvement from the 4.8-month historical mPFS with axitinib alone. Patients treated with mavorixafor + axitinib in the subgroup with immediate prior IO therapy (n=18) had an ORR of 61% and an increased mPFS of 11.6 months. In addition, eight of the 65 patients remain on the combination therapy as of February 2020 with durations of treatment of 26-44 months or longer. Results suggest mavorixafor may enhance clinical response to axitinib and other TKIs that target tumor angiogenesis, as well as immunotherapy agents.

Future development and potential commercialization of mavorixafor in ccRCC and other potential immuno-oncology indications will be pursued only as part of a potential strategic collaboration.

Earlier-Stage Candidates and Research Efforts

X4P-003: We are developing a second-generation CXCR4 antagonist that is at the stage of candidate drug nomination designed to have an enhanced pharmacokinetic profile relative to mavorixafor, potentially enabling improved patient compliance and ease of use to better serve patients suffering from chronic rare diseases.

X4P-002: We are also developing X4P-002, a CXCR4 antagonist designed to cross the blood-brain barrier with a potential to provide therapeutic exposures necessary to treat glioblastoma multiforme, or GBM. GBM is the one of the most aggressive forms of brain cancer with a five-year overall survival rate of less than 10%. GBM accounts for about 15% of brain cancers. CXCR4 is an important mediator of malignant cell invasiveness in in vitro and in vivo studies. We believe that X4P-002 is the only oral CXCR4 antagonist product candidate that has been shown, in preclinical studies, to penetrate the blood-brain barrier in levels that exceed the targeted levels required for an optimized anti-cancer effect in animal studies.

Research Efforts: We are expanding our research facility in Vienna, Austria to further expand our insights into CXCR4 biology, translational science and early stage discovery in primary immunodeficiencies.

Arsanis Programs

We have undertaken a strategic review of our development programs focused on applying monoclonal antibody, or mAb, immunotherapies to address serious infectious diseases that were being developed by Arsanis prior to the Merger. As part of this review, we have discontinued our ASN100 *Staphylococcus aureus pneumonia* program. We entered into several collaborations and out-licensing arrangements, with respect to continued development of our ASN200 for *Escherichia coli* and ASN300 for *Klebsiella pneumonia* programs, both of which have been exclusively licensed to Bravo Biosciences, LLC, or Bravos, and our ASN500 respiratory syncytial virus, or RSV, program, which has been exclusively licensed to Evotec.

Competition

The pharmaceutical and biotechnology industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. We face potential competition from many different sources, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions, 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. Other firms also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient enrollment for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. 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. Smaller or early-stage companies may also prove to be significant competitors with us, particularly through collaborative arrangements with large and established companies.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize therapeutics 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 marketing approvals for their products more rapidly than we may obtain approval for our products, 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 because in some cases insurers or other third-party payors, including government programs, seek to encourage the use of generic products. This may have the effect of making branded products less attractive, from a cost perspective, to buyers.

We are aware of other companies that are developing CXCR4 inhibitors that are in a similar stage of development as mavorixafor, including Eli Lilly, Pfizer, Bristol-Myers Squibb, or BMS, BioLineRx, Noxxon, Upsher-Smith, Polyphor and Glycomimetics. To our knowledge, there do not appear to be any competitors with programs in development for WHIM syndrome or SCN. With respect to WM, the Dana Farber Cancer Institute has initiated a trial to study the BMS CXCR4 antibody (IV infusion) in the treatment of WM patients with CXCR4 mutations.

In WM, there are several treatment approaches currently being developed, including targeted therapies and immunotherapies (as monotherapies and combination therapies), chemotherapy, stem cell transplantation, and cancer vaccines. Our principal competitors in ccRCC include Pfizer, Novartis, BMS, and Merck. To our knowledge, only X4 and BMS have therapies in development that directly target the 30-40% of WM patients with the double mutations (MYD88 and CXCR4) and only X4 is developing an oral therapy. In glioblastoma, our principal competitors include Genentech/Roche and BMS.

Manufacturing

We do not own or operate, and currently have no plans to establish, manufacturing facilities for the production of clinical or commercial quantities of mavorixafor or any of our other product candidates. We currently rely, and expect to continue to rely, on third parties for the manufacture of our product candidates and any products that we may develop.

We currently have a master services agreement, or the Aptuit Agreement, in place with Aptuit (Oxford) Ltd, or Aptuit, pursuant to which Aptuit develops and manufactures the active pharmaceutical ingredient, or API, for mavorixafor. We use this mavorixafor drug substance in our Phase 3 clinical trial for the treatment of WHIM and in our other clinical trials. Aptuit is our sole supplier for mavorixafor drug substance and is currently manufacturing mavorixafor drug substance at a commercially relevant scale to support our ongoing and anticipated future clinical trials.

The term of the Aptuit Agreement expires on February 19, 2021 unless terminated by us and/or Aptuit as follows: (i) by mutual agreement of the parties; (ii) by us, if there is a material breach by the other party that remains uncured; (iii) by us, in the event of insolvency or bankruptcy of Aptuit; or (iv) by either party, upon 30 days' written notice.

We also have a master services agreement in place with Mayne Pharma Inc., or Mayne Pharma, which is our sole manufacturer to provide fill and finish services for the final drug product formulation of mavorixafor for use in our clinical trials. The term of the master services agreement expires on September 10, 2023, and may be terminated by (1) us upon 30 days-notice to Mayne Pharma or (2) by either party following a material breach by the other party that remains uncured for 30 days.

We obtain the supplies of our API and drug products from Aptuit and Mayne pursuant to typical industry standard clinical supply agreements. We believe that both API and drug product manufacturers have the capability and capacity to manufacture currently projected clinical trial supply and commercial volumes of mavorixafor and we are engaged in active discussions with both parties to plan anticipated commercial manufacturing arrangements. We obtain the supplies of our product candidates from these

manufacturers under master services contracts and specific work orders. However, we do not have long-term supply arrangements in place. We do not currently have arrangements in place for redundant supply or a second source for API for mavorixafor. If any of our current manufacturers becomes unavailable to us for any reason, we believe that there are a number of potential replacements, although we might incur some delay in identifying and qualifying such replacements.

License Agreements

License Agreement with Genzyme

In July 2014, we entered into a license agreement with Genzyme, or the Genzyme Agreement, pursuant to which we were granted an exclusive license to certain patent applications and other intellectual property owned or controlled by Genzyme related to the CXCR4 receptor to develop and commercialize products containing licensed compounds (including but not limited to mavorixafor) for all therapeutic, prophylactic and diagnostic uses with the exception of autologous and allogenic human stem cell therapy. Genzyme has retained the exclusive right to use the intellectual property licensed to us in specific indications related to Genzyme's product Mozobil® and allogenic/autologous hematopoietic stem cell transplantation treatments. Genzyme has also retained the non-exclusive right to conduct preclinical research involving compounds in any field, including any fields licensed to us, but has not retained rights to conduct any clinical development or commercialization of those compounds identified in the agreement in any of the fields licensed to us. We are primarily responsible for the preparation, filing, prosecution and maintenance of all patent applications and patents covering the intellectual property licensed to us under the agreement at our sole expense.

Under the terms of the Genzyme Agreement, we are obligated to use commercially reasonable efforts to develop and commercialize licensed products for use in the field in the United States and at least one other major market country. We have the right to grant sublicenses of the licensed rights that cover mavorixafor to third parties. If we wish to grant a sublicense to any licensed product other than mavorixafor, we are obligated to first offer the sublicense to Genzyme. If Genzyme expresses written interest for the sublicense, then we will negotiate exclusively with Genzyme for a certain stated period to obtain a license to such rights, after which Genzyme shall have no further rights with respect to such licensed product and we will be free to negotiate a sublicense with respect to such licensed product with any third party.

In accordance with the Genzyme Agreement, X4 issued Genzyme 107,364 shares of common stock following its Series A financing. No further issuance of shares is required under the terms of the license.

We are obligated to pay Genzyme milestone payments in the aggregate amount of up to $25,000,000, contingent upon our achievement of certain late-stage regulatory and sales milestones with respect to licensed products. In addition,X4 was required to make a one-time milestone payment to Genzyme upon the consummation by X4 of a change of control transaction in an amount equal to 5.5% of the consideration paid to its equity holders, other than Genzyme, in connection with such change of control transaction, after deducting its outstanding debt obligations and the aggregate cash investments made by our equity holders prior to the closing of the change of control transaction. The Merger qualified as a change of control event, as defined in the license agreement, but resulted in no payment being due to Genzyme under the license agreement.

We are obligated under the Genzyme agreement to pay Genzyme tiered royalties based on net sales of licensed products that we commercialize under the Genzyme agreement. Our obligation to pay royalties for each licensed product expires on a country-by-country basis on the latest of (i) the expiration of licensed patent rights that cover that licensed product in that country, (ii) the expiration of regulatory exclusivity in that country and (iii) ten years after the first commercial sale of such licensed product in that country. Royalty rates are subject to reduction under the agreement in specified circumstances, including in any country if we are required to obtain a license from any third party to the extent our patent rights might infringe the third party's patent rights, if a licensed product is not covered by a valid claim in that country or if sales of generic products reach certain thresholds in that country. If we enter into a sublicense under the Genzyme agreement, we will be obligated to pay Genzyme a percentage of certain upfront, maintenance fees, milestone payments and royalty payments paid to us by the sublicensee.

The term of the Genzyme Agreement will continue until the later of the expiration of the last to expire valid claim of the patents licensed under the agreement that cover any licensed product, the expiration of regulatory exclusivity applicable to any licensed product and 10 years from the date of first commercial sale of any licensed product. Either we or Genzyme may terminate the Genzyme Agreement in the event of the bankruptcy or uncured material breach by the other party. Genzyme may terminate the Genzyme Agreement if we or our affiliates initiate a patent challenge of the patents licensed under the agreement. We may terminate the Genzyme Agreement immediately upon notice to Genzyme if we reasonably believe that the development or

commercialization of a licensed compound or product under the Genzyme agreement would result in a material safety issue for patients.

License Agreement with Georgetown University

In December 2016, we entered into a license agreement with the Georgetown University, or Georgetown, pursuant to which we obtained an exclusive, worldwide license to practice certain methods, and to make, have made, use, sell, offer for sale and import products, covered by licensed patent rights co-owned by Georgetown. The rights licensed to us are for all therapeutic, prophylactic and diagnostic uses in all disease indications in humans and animals. We have the right to grant sublicenses of the licensed rights to third parties to the extent consistent with the terms of the Georgetown agreement.

Under the terms of the Georgetown agreement we paid a one-time only, upfront fee of $50,000, and we may be required to pay milestone payments of up to an aggregate of $800,000 related to commercial sales of a licensed product. We are responsible for all patent prosecution costs incurred with respect to the licensed patents. We are obligated under the agreement to use commercially reasonable efforts to develop and commercialize licensed product, to make licensed product reasonably available to the public, to obtain government approvals for licensed product and to market licensed product in quantities sufficient to meet the market demand.

The term of the Georgetown agreement will continue until the expiration of the last valid claim within the patent rights covering the licensed products. Georgetown may terminate the Georgetown agreement or convert our license to non-exclusive in the event (i) we fail to pay any amount and fail to cure such failure within 30 days after receipt of notice, (ii) we default in our obligation to obtain and maintain insurance and fail to remedy such breach within 45 days after receipt of notice, (iii) we declare insolvency or bankruptcy or (iv) we materially default in the performance of any material obligations under the Georgetown agreement which is not cured within a certain period from the date of written notice of such default. We may terminate the Georgetown agreement at any time upon at least 60 days' written notice.

License Agreement with Beth Israel Deaconess Medical Center

In December 2016, we entered into a license agreement with Beth Israel Deaconess Medical Center, or BIDMC, pursuant to which we obtained an exclusive, worldwide license to make, have made, use, sell, offer for sale and import of licensed products and certain processes covered by licensed patent rights co-owned by BIDMC and a nonexclusive royalty-free right to use certain information pertaining to any invention claimed in the licensed patents that is owned by BIDMC to develop, make, have made, use, have used, sell, have sold and commercialize such licensed products and processes. The rights licensed to us are for all fields of use. We have the right to grant sublicenses of the licensed rights to third parties to the extent consistent with the terms of the agreement.

Under the terms of the BIDMC agreement we paid a one-time only, upfront fee of $20,000 and we are responsible for all future patent prosecution costs.

The term of the BIDMC agreement will continue until the expiration of the last valid claim within the patent rights covering the licensed product. BIDMC may terminate the agreement in the event (i) we fail to pay any amount and fail to cure such failure within 15 days after receipt of notice, (ii) the insurance coverage that we are obligated to maintain under the agreement is terminated and we fail to obtain replacement insurance within a certain period of time following notice to BIDMC, or (iii) we declare insolvency or bankruptcy. In addition, if we are in material breach of any material provisions of the BIDMC agreement and fail to remedy such breach within 60 days after receipt of notice, BIDMC may terminate the BIDMC agreement or terminate any licenses granted under the agreement with respect to the country or countries in which such material breach has occurred. We may terminate the agreement at any time upon at least 90 days' written notice.

Abbisko Agreement

In July 2019, we entered into a license agreement with Abbisko Therapeutics Co., Ltd., or Abbisko. Under the terms of the agreement, we granted Abbisko the exclusive right to develop, manufacture and commercialize mavorixafor in mainland China, Taiwan, Hong Kong and Macau. The agreement provides Abbisko with the exclusive rights in this territory to develop and commercialize mavorixafor in combination with checkpoint inhibitors or other agents in oncology indications. Pancreatic cancer, ovarian cancer and triple negative breast cancer will be explored initially. We retain the full rest-of-world rights to develop and commercialize mavorixafor outside of Greater China for all indications and the ability to utilize data generated pursuant to the Abbisko collaboration for rest-of-world development. In addition, Abbisko has the right of first refusal if we determine to pursue additional products in the Abbisko Territory, as defined in the agreement. We have agreed to enter into separate agreements

whereby we would provide Abbisko with a clinical supply and, if the product is commercialized in the territory licensed by Abbisko, a commercial supply of the licensed compound.

Pursuant to the agreement with Abbisko, upon the closing of a qualified financing, as defined in the agreement, Abbisko will make a one-time, non-refundable, non-creditable financial milestone payment in the low single-digit millions to us. We are also eligible to receive potential development and regulatory milestone payments and potential commercial milestone payments based on annual net sales of licensed products. Upon commercialization of mavorixafor in the Abbisko Territory, we are eligible to receive a tiered royalty, with a percentage range in the low double-digits, on net sales of approved licensed products. Abbisko is obligated to use commercially reasonable efforts to develop and commercialize mavorixafor in the Abbisko Territory. Abbisko has responsibility for all activities and costs associated with the further development, manufacture and commercialization of mavorixafor in the Abbisko Territory.

Intellectual Property

Our ability to commercialize our product candidates depends in large part on our ability to obtain and maintain intellectual property protection for our product candidates, including mavorixafor, and our preclinical compounds and core technologies. Our policy is to seek to protect our intellectual property position by, among other methods, filing U.S. and foreign patent applications related to the technology, inventions and improvements that are important to the development and implementation of our business strategy. We also rely on trade secrets, know-how and continuing technological innovation to develop and maintain our proprietary position.

We file patent applications directed to our product candidates, preclinical compounds and related technologies to establish intellectual property positions on these compounds and their uses in disease. As of December 31, 2019, we owned or exclusively licensed 13 issued U.S. patents, 12 pending U.S. non-provisional patent applications, one pending U.S. provisional patent application, and approximately 132 PCT and foreign patents and patent applications in the following foreign jurisdictions: Belgium, Brazil, Canada, China, European Patent Office, France, Germany, Great Britain, Hong Kong, India, Ireland, Italy, Israel, Japan, Lichtenstein, Mexico, Netherlands, Spain, Sweden and Switzerland.

As of December 31, 2019, our in-licensed intellectual property portfolio for mavorixafor included two issued U.S. patents and one pending U.S. patent application directed to compositions of matter for mavorixafor, which is expected to expire in December 2022 excluding possible patent term extensions of up to an additional five years. The intellectual property portfolio for mavorixafor also included one issued U.S. patent with claims directed to a crystalline salt form of mavorixafor, one issued U.S. patent directed to pharmaceutical compositions of mavorixafor in unit dosage form, and four issued U.S. patents directed to methods of making mavorixafor and key intermediates. We also had four issued U.S. patents directed to compositions and methods of making chemical compounds related to the X4P-001 program. Approximately 85 corresponding PCT and foreign patents and patent applications directed to compositions of matter and related chemical compounds as well as methods of making and methods of use were issued or pending. All of the above patents and patent applications were exclusively licensed to us pursuant to the terms of the Genzyme license agreement.

Additionally, we have filed our own patent applications with respect to the mavorixafor and X4P-002 product candidates. Some of these patent applications are co-owned with Genzyme, BIDMC or Georgetown, with their rights exclusively licensed to X4. As of December 31, 2019, our independently generated intellectual property portfolio included eight pending U.S. non-provisional patent applications, one pending U.S. provisional patent application, and approximately 31 pending PCT and foreign patent applications related to our mavorixafor clinical programs in cancer and primary immunodeficiencies; and three pending U.S. non-provisional patent applications, one pending U.S. provisional patent application and 16 pending PCT and foreign patent applications related to our preclinical compounds and X4P-002 in glioblastoma. Patents issuing from these applications, if any, are expected to expire between 2036 and 2039.

The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries, including the United States, the patent term is 20 years from the earliest filing date of a non-provisional patent application. In the United States, a patent’s term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the U.S. Patent and Trademark Office, or the USPTO, in examining and granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier filed patent. The term of a U.S. patent that covers a drug or biological product may also be eligible for patent term extension when approval from the FDA is granted, provided statutory and regulatory requirements are met. In the future, if our product candidates receive approval from the FDA or foreign regulatory authorities, we expect to apply for patent term extensions on issued patents covering those products, depending upon the length of the clinical

trials for each drug and other factors. There can be no assurance that any of our pending patent applications will issue or that we will benefit from any patent term extension or other favorable adjustment to the term of any of our patents.

As with other biotechnology and pharmaceutical companies, our ability to maintain and solidify our proprietary and intellectual property position for our product candidates, including mavorixafor, and our preclinical compounds, and our core technologies will depend on our success in obtaining effective patent claims and enforcing those claims if granted. However, patent applications that we may file or license from third parties may not result in the issuance of patents. We also cannot predict the breadth of claims that may be allowed or enforced in our patents. Any issued patents that we may receive in the future may be challenged, invalidated or circumvented. For example, prior to March 16, 2013, in the United States, patent applications were subject to a "first to invent" rule of law. Applications filed after March 16, 2013 (except for certain applications claiming the benefit of earlier-filed applications) are subject to a "first to file" rule of law.

Discoveries reported in the scientific literature often lag the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. We cannot be certain that any existing or future application will be subject to the "first to file" or "first to invent" rule of law, that we were the first to make the inventions claimed in our existing patents or pending patent applications subject to the prior laws, or that we were the first to file for patent protection of such inventions subject to the new laws. If third parties prepare and file patent applications in the United States that also claim technology we have claimed in our patents or patent applications, we may have to participate in interference proceedings in the USPTO to determine priority of invention, which could result in substantial costs to us, even if the eventual outcome is favorable to us. In addition, because of the extensive time required for clinical development and regulatory review of a product candidate we may develop, it is possible that, before any of our product candidates can be commercialized, any related patent may expire or remain in force for only a short period following commercialization, thereby reducing any advantage of any such patent.

In addition to patents, we rely upon unpatented trade secrets, know-how, and continuing technological innovation to develop and maintain our competitive position. We seek to protect our proprietary information, in part, by using confidentiality agreements with our collaborators, scientific advisors, employees and consultants, and invention assignment agreements with our employees. We also have agreements requiring assignment of inventions with selected consultants, scientific advisors and collaborators. The confidentiality agreements are designed to protect our proprietary information and, in the case of agreements or clauses requiring invention assignment, to grant us ownership of technologies that are developed under those agreements.

Government Regulation and Product Approval

The FDA Approval Process

In the United States, pharmaceutical products are subject to extensive regulation by the FDA. The Federal Food, Drug, and Cosmetic Act, or the FDCA, and other federal and state statutes and regulations, govern, among other things, the research, development, testing, manufacture, storage, recordkeeping, approval, labeling, promotion and marketing, distribution, post-approval monitoring and reporting, sampling, and import and export of pharmaceutical products. Failure to comply with applicable U.S. requirements may subject a company to a variety of administrative or judicial sanctions, such as imposition of clinical holds, refusal by the FDA to approve pending NDAs, warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement, civil penalties and criminal prosecution.

Pharmaceutical product development in the United States typically involves preclinical or other nonclinical laboratory and animal tests and the submission to the FDA of an IND, which must become effective before clinical testing may commence. For commercial approval, the sponsor must submit adequate tests by all methods reasonably applicable to show that the drug is safe for use under the conditions prescribed, recommended or suggested in the proposed labeling. The sponsor must also submit substantial evidence, generally consisting of adequate, well-controlled clinical trials to establish that the drug will have the effect it purports or is represented to have under the conditions of use prescribed, recommended or suggested in the proposed labeling. In certain cases, the FDA may determine that a drug is effective based on one clinical study plus confirmatory evidence. Satisfaction of the FDA pre-market approval requirements typically takes many years and the actual time required may vary substantially based upon the type, complexity and novelty of the product or disease.

Nonclinical tests include laboratory evaluation of product chemistry, formulation and toxicity, as well as animal studies to assess the characteristics and potential safety and efficacy of the product. The conduct of the nonclinical tests must comply with federal requirements, including the FDA's good laboratory practices regulations and the U.S. Department of Agriculture's, or USDA's,

regulations implementing the Animal Welfare Act. The results of nonclinical testing are submitted to the FDA as part of an IND along with other information, including information about product chemistry, manufacturing and controls, and a proposed clinical trial protocol. Long-term nonclinical tests, such as animal studies of reproductive toxicity and carcinogenicity, may continue after the IND is submitted.

A 30-day waiting period after the submission of each IND is required prior to the commencement of clinical testing in humans. If the FDA has not imposed a clinical hold on the IND or otherwise commented or questioned the IND within this 30-day period, the clinical trial proposed in the IND may begin.

Clinical trials involve the administration of the investigational new drug to healthy volunteers or patients under the supervision of a qualified investigator. Clinical trials must be conducted: (i) in compliance with federal regulations, (ii) in compliance with GCP, an international standard meant to protect the rights and health of patients and to define the roles of clinical trial sponsors, administrators and monitors, and (iii) under protocols detailing the objectives of the trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. Each protocol involving testing on U.S. patients and subsequent protocol amendments must be submitted to the FDA as part of the IND.

The FDA may order the temporary, or permanent, discontinuation of a clinical trial at any time or impose other sanctions if it believes that the clinical trial either is not being conducted in accordance with the FDA requirements or presents an unacceptable risk to the clinical trial patients. The trial protocol and informed consent information for patients in clinical trials must also be submitted to an institutional review board, or IRB, at each site where a trial will be conducted for approval. An IRB may also require the clinical trial at the site to be halted, either temporarily or permanently, for failure to comply with the IRB's requirements or may impose other conditions.

Clinical trials to support NDAs for marketing approval are typically conducted in three sequential phases, but the phases may overlap. In general, in Phase 1, the initial introduction of the drug into healthy human volunteers or, in some cases, patients, the drug is tested to assess metabolism, pharmacokinetics, pharmacological actions, side effects associated with increasing doses and, if possible, early evidence of effectiveness. Phase 2 usually involves trials in a limited patient population to determine the effectiveness of the drug for a particular indication, dosage tolerance and optimum dosage, and to identify common adverse effects and safety risks. If a compound demonstrates evidence of effectiveness and an acceptable safety profile in Phase 2 evaluations, Phase 3 trials are undertaken to obtain the additional information about clinical efficacy and safety in a larger number of patients, typically at geographically dispersed clinical trial sites, to permit the FDA to evaluate the overall benefit-risk relationship of the drug and to provide adequate information for the labeling of the drug. In most cases, the FDA requires two adequate and well-controlled Phase 3 clinical trials to demonstrate the efficacy of the drug. The FDA may, however, determine that a drug is effective based on one clinical trial plus confirmatory evidence. Only a small percentage of investigational drugs complete all three phases and obtain marketing approval. In some cases, the FDA may require post-market studies, known as Phase 4 studies, to be conducted as a condition of approval to gather additional information on the drug's effect in various populations and any side effects associated with long-term use. Depending on the risks posed by the drugs, other post-market requirements may be imposed.

After completion of the required clinical testing, an NDA is prepared and submitted to the FDA. FDA approval of the NDA is required before marketing of the product may begin in the United States. The NDA must include the results of all preclinical, clinical, and other testing and a compilation of data relating to the product's pharmacology, chemistry, manufacture, and controls. The cost of preparing and submitting an NDA is substantial. Under federal law, the submission of most NDAs is additionally subject to a substantial application user fee, subject to certain exceptions and waivers, such as for orphan-designated drugs.

The FDA has 60 days from its receipt of an NDA to determine whether the application will be accepted for filing based on the agency's threshold determination that it is sufficiently complete to permit substantive review. Once the submission is accepted for filing, the FDA begins an in-depth review. Under the statute and implementing regulations, the FDA has 180 days (the initial review cycle) from the date of filing to issue either an approval letter or a complete response letter, unless the review period is adjusted by mutual agreement between the FDA and the applicant or as a result of the applicant submitting a major amendment. In practice, the performance goals established pursuant to the Prescription Drug User Fee Act have effectively extended the initial review cycle beyond 180 days. The FDA's current performance goals call for the FDA to complete review of 90% of standard (non-priority) NDAs within 10 months of receipt and within six months for priority NDAs, but two additional months are added to standard and priority NDAs for a new molecular entity, or NME.

The FDA may also refer applications for novel drug products, or drug products that present difficult questions of safety or efficacy, to an advisory committee, which is 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 recommendation of an advisory committee, but it generally follows such recommendations. Before approving an NDA, the FDA will typically inspect one or more clinical sites to assure compliance with GCP. Additionally, the FDA will inspect the facility or the facilities at which the drug is manufactured. The FDA will not approve the product unless compliance with current GMP is satisfactory and the NDA contains data that provide substantial evidence that the drug is safe and effective in the indication studied.

After the FDA evaluates the NDA and the manufacturing facilities, it issues either an approval letter or a complete response letter. 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, or 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 90% of resubmissions within two to six months depending on the type of information included.

An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. As a condition of NDA approval, the FDA may require a risk evaluation and mitigation strategy, or REMS, to help ensure that the benefits of the drug outweigh the potential risks. REMS can include medication guides, communication plans for health care professionals, and elements to assure safe use, or ETASU. ETASU can include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring, and the use of patient registries. The requirement for a REMS can materially affect the potential market and profitability of the drug. Moreover, product approval may require substantial post-approval testing and surveillance to monitor the drug's safety or efficacy. Once granted, product approvals may be withdrawn if compliance with regulatory standards is not maintained or problems are identified following initial marketing.

Disclosure of Clinical Trial Information

Sponsors of clinical trials of certain FDA-regulated products, including prescription drugs, are required to register and disclose certain clinical trial information on a public website maintained by the U.S. National Institutes of Health. Information related to the product, patient population, phase of investigation, study sites and investigator, and other aspects of the clinical trial is made public as part of the registration. Sponsors are also obligated to disclose the results of these trials after completion. Disclosure of the results of these trials can be delayed for up to two years if the sponsor certifies that it is seeking approval of an unapproved product or that it will file an application for approval of a new indication for an approved product within one year. Competitors may use this publicly available information to gain knowledge regarding the design and progress of the development programs.

The Hatch-Waxman Act

Orange Book Listing

In seeking approval for a drug through an NDA, applicants are required to list with the FDA each patent whose claims cover the applicant's 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 generic competitors in support of approval of an abbreviated new drug application, or ANDA. An ANDA provides for marketing of a drug product that has the same active ingredients in the same strengths and dosage form as the listed drug and has been shown through bioequivalence testing to be bioequivalent to the listed drug. Other than the requirement for bioequivalence testing, ANDA applicants are not required to conduct, or submit results of, pre-clinical or clinical tests to prove the safety or effectiveness of their drug product. Drugs approved in this way are considered to be therapeutically equivalent to the listed drug, 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 in accordance with state law.

The ANDA applicant is required to certify to the FDA concerning any patents listed for the approved product in the FDA's Orange Book. Specifically, the applicant must certify that: (i) the required patent information has not been filed; (ii) the listed patent has expired; (iii) the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or (iv) the listed patent is invalid or will not be infringed by the new product. The ANDA applicant may also elect to submit a section viii statement, certifying that its proposed ANDA labeling does not contain (or carves out) any language regarding the patented method-of-use, rather than certify to a listed method-of-use patent.

If the applicant does not challenge the listed patents, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired.

A certification that the new product will not infringe the already approved product's listed patents, or that such patents are invalid, is called a Paragraph IV certification. 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 of the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months, expiration of the patent, settlement of the lawsuit, or a decision in the infringement case that is favorable to the ANDA applicant.

The ANDA application also will not be approved until any applicable non-patent exclusivity listed in the Orange Book for the referenced product has expired.

Exclusivity
Upon NDA approval of a new chemical entity, or NCE, which is a drug that contains no active moiety that has been approved by the FDA in any other NDA, that drug receives five years of marketing exclusivity during which time the FDA cannot receive any ANDA or 505(b)(2) application seeking approval of a drug that references a version of the NCE drug. Certain changes to a drug, such as the addition of a new indication to the package insert, are associated with a three-year period of exclusivity during which the FDA cannot approve an ANDA or 505(b)(2) application that includes the change.

An ANDA or 505(b)(2) application may be submitted one year before NCE exclusivity expires if a Paragraph IV certification is filed. If there is no listed patent in the Orange Book, there may not be a Paragraph IV certification and thus no ANDA or 505(b)(2) application may be filed before the expiration of the exclusivity period.

For a botanical drug, the FDA may determine that the active moiety is one or more of the principal components or the complex mixture as a whole. This determination would affect the utility of any five-year exclusivity as well as the ability of any potential generic competitor to demonstrate that it is the same drug as the original botanical drug.

Five-year and three-year exclusivities do not preclude FDA approval of a 505(b)(1) application for a duplicate version of the drug during the period of exclusivity, provided that the 505(b)(1) applicant conducts or obtains a right of reference to all of the preclinical studies and adequate and well controlled clinical trials necessary to demonstrate safety and effectiveness.

Patent Term Extension
After NDA approval, owners of relevant drug patents may apply for up to a five-year patent extension. The allowable patent term extension is calculated as half of the drug's testing phase—the time between IND submission and NDA submission—and all of the review phase—the time between NDA submission and approval up to a maximum of five years. The time can be shortened if the FDA determines that the applicant did not pursue approval with due diligence. The total patent term after the extension may not exceed 14 years.

For patents that might expire during the application phase, the patent owner may request an interim patent extension. An interim patent extension increases the patent term by one year and may be renewed up to four times. For each interim patent extension granted, the post-approval patent extension is reduced by one year. The director of the USPTO must determine that approval of the drug covered by the patent for which a patent extension is being sought is likely. Interim patent extensions are not available for a drug for which an NDA has not been submitted.

Advertising and Promotion
Once an NDA is approved, a product will be subject to certain post-approval requirements. For instance, the FDA closely regulates the post-approval marketing and promotion of drugs.

Drugs may be marketed only for the approved indications and in accordance with the provisions of the approved labeling. Changes to some of the conditions established in an approved application, including changes in indications, labeling, or manufacturing processes or facilities, require submission and FDA approval of a new NDA or NDA supplement before the change can be implemented. An NDA supplement for a new indication typically requires clinical data similar to that in the

original application, and the FDA uses the same procedures and actions in reviewing NDA supplements as it does in reviewing NDAs.

Adverse Event Reporting and GMP Compliance

Adverse event reporting and submission of periodic reports is required following FDA approval of an NDA. The FDA also may require post-marketing testing, known as Phase 4 testing, require a REMS special communications regarding the safety of the drug or heightened surveillance to monitor the effects of an approved product, or may place conditions on an approval that could restrict the distribution or use of the product. In addition, quality control, drug manufacture, packaging, and labeling procedures must continue to conform to GMP after approval. Drug manufacturers and certain of their subcontractors are required to register their establishments with the FDA and certain state agencies. Registration with the FDA subjects entities to periodic unannounced inspections by the FDA, during which the agency inspects manufacturing facilities to assess compliance with GMP. Accordingly, manufacturers must continue to expend time, money and effort in the areas of production and quality control to maintain compliance with GMP. Regulatory authorities may withdraw product approvals or request product recalls if a company fails to comply with regulatory standards, if it encounters problems following initial marketing or if previously unrecognized problems are subsequently discovered.

Pediatric Exclusivity and Pediatric Use

The Best Pharmaceuticals for Children Act, or BPCA, provides NDA holders a six-month period of exclusivity attached to any other exclusivity listed with FDA—patent or non-patent—for a drug if certain conditions are met. Conditions for pediatric exclusivity include a determination by the FDA that information relating to the use of a new drug in the pediatric population may produce health benefits in that population; a written request by the FDA for pediatric studies; and agreement by the applicant to perform the requested studies and the submission to the FDA, completion of the studies in accordance with the written request, and the acceptance by the FDA of the reports of the requested studies within the statutory timeframe. Applications under the BPCA are treated as priority applications.

In addition, under the Pediatric Research Equity Act, or PREA, NDAs or supplements to NDAs must contain data 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 drug is safe and effective, unless the sponsor has received a deferral or waiver from the FDA. Unless otherwise required by regulation, PREA does not apply to any drug for an indication for which orphan designation has been granted. The sponsor or the FDA may request a deferral of pediatric studies for some or all of the pediatric subpopulations. A deferral may be granted for several reasons, including a finding that the drug is ready for approval for use in adults before pediatric studies are complete or that additional safety or effectiveness data need to be collected before the pediatric studies begin. Under PREA, the FDA must send a non-compliance letter requesting a response within 45 days to any sponsor that fails to submit the required assessment, fails to keep a deferral current or fails to submit a request for approval of a pediatric formulation.

Orphan Drugs

Under the Orphan Drug Act, the FDA may grant orphan drug designation to drugs intended to treat a rare disease or condition—generally a disease or condition that affects fewer than 200,000 individuals in the United States (or affects more than 200,000 in the United States and for which there is no reasonable expectation that the cost of developing and making available in the United States a drug for such disease or condition will be recovered from sales of such drug 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 disease, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity. If the FDA designates an orphan drug based on a finding of clinical superiority, the FDA must provide a written notification to the sponsor that states the basis for orphan designation, including “any plausible hypothesis” relied upon by the FDA. The FDA must also publish a summary of its clinical superiority findings upon granting orphan drug exclusivity based on clinical superiority.

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.

Breakthrough Designation
A product can be designated as a breakthrough therapy by FDA if it is intended to treat a serious or life-threatening condition and preliminary clinical evidence indicates that it may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. A sponsor may request that a product candidate be designated as a breakthrough therapy concurrently with the submission of an IND or any time before an end-of-Phase-2 meeting, and the FDA must determine if the product candidate qualifies for breakthrough therapy designation within 60 days of receipt of the sponsor's request. If so designated, the FDA shall act to expedite the development and review of the product's marketing application, including by meeting with the sponsor throughout the product's development, providing timely advice to the sponsor to ensure that the development program to gather pre-clinical and clinical data is as efficient as practicable, involving senior managers and experienced review staff in a cross-disciplinary review, assigning a cross-disciplinary project lead for the FDA review team to facilitate an efficient review of the development program and to serve as a scientific liaison between the review team and the sponsor, and taking steps to ensure that the design of the clinical trials is as efficient as practicable.

Europe/Rest of World Government Regulation
In addition to regulations in the United States, we are and will be subject, either directly or through our distribution partners, to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of our products, if approved.

Whether or not we obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in non-U.S. countries prior to the commencement of clinical trials or marketing of the product in those countries.

In the European Union, medicinal products are subject to extensive pre- and post-marketing regulation by regulatory authorities at both the European Union and national levels. Additional rules also apply at the national level to the manufacture, import, export, storage, distribution and sale of controlled substances. In many E.U. member states, the regulatory authority responsible for medicinal products is also responsible for controlled substances. Responsibility is, however, split in some member states. Generally, any company manufacturing or distributing a medicinal product containing a controlled substance in the European Union will need to hold a controlled substances license from the competent national authority and will be subject to specific record-keeping and security obligations. Separate import or export certificates are required for each shipment into or out of the member state.

Clinical Trials and Marketing Approval
Certain countries outside of the United States have a process that requires the submission of a clinical trial application much like an IND prior to the commencement of human clinical trials. In Europe, for example, a clinical trial application, or CTA, must be submitted to the competent national health authority and to independent ethics committees in each country in which a company intends to conduct clinical trials. Once the CTA is approved in accordance with a country's requirements and a company has received favorable ethics committee approval, clinical trial development may proceed in that country.

The requirements and process governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country, even though there is already some degree of legal harmonization in the European Union member states resulting from the national implementation of underlying E.U. legislation. In all cases, the clinical trials must be conducted in accordance with the International Conference on Harmonization, or ICH, guidelines on GCP and other applicable regulatory requirements.

To obtain regulatory approval to place a drug on the market in the European Union, we must submit a marketing authorization application. This application is similar to the NDA in the United States, with the exception of, among other things, country-specific document requirements. All application procedures require an application in the common technical document, or CTD, format, which includes the submission of detailed information about the manufacturing and quality of the product, and non-clinical and clinical trial information. Drugs can be authorized in the European Union by using (i) the centralized authorization procedure, (ii) the mutual recognition procedure, (iii) the decentralized procedure or (iv) national authorization procedures.

The European Commission created the centralized procedure for the approval of human drugs to facilitate marketing authorizations that are valid throughout the European Union and, by extension (after national implementing decisions) in Iceland, Liechtenstein and Norway, which, together with the E.U. member states, comprise the European Economic Area, or EEA. Applicants file marketing authorization applications with the EMA, where they are reviewed by a relevant scientific committee, in most cases the Committee for Medicinal Products for Human Use, or CHMP. The EMA forwards CHMP opinions to the European Commission, which uses them as the basis for deciding whether to grant a marketing authorization. This procedure results in a single marketing authorization granted by the European Commission that is valid across the European Union, as well as in Iceland, Liechtenstein and Norway. The centralized procedure is compulsory for human drugs that are: (i) derived from biotechnology processes, such as genetic engineering, (ii) contain a new active substance indicated for the treatment of certain diseases, such as HIV/AIDS, cancer, diabetes, neurodegenerative diseases, autoimmune and other immune dysfunctions and viral diseases, (iii) officially designated "orphan drugs" (drugs used for rare human diseases) and (iv) advanced-therapy medicines, such as gene-therapy, somatic cell-therapy or tissue-engineered medicines. The centralized procedure may at the voluntary request of the applicant also be used for human drugs which do not fall within the above-mentioned categories if the CHMP agrees that (a) the human drug contains a new active substance not yet approved on November 20, 2005; (b) it constitutes a significant therapeutic, scientific or technical innovation or (c) authorization under the centralized procedure is in the interests of patients at the E.U. level.

Under the centralized procedure in the European Union, the maximum timeframe for the evaluation of a marketing authorization application by the EMA is 210 days (excluding clock stops, when additional written or oral information is to be provided by the applicant in response to questions asked by the CHMP), with adoption of the actual marketing authorization by the European Commission thereafter. Accelerated evaluation might be granted by the CHMP in exceptional cases, when a medicinal product is expected to be of a major public health interest from the point of view of therapeutic innovation, defined by three cumulative criteria: the seriousness of the disease to be treated, the absence of an appropriate alternative therapeutic approach, and anticipation of exceptional high therapeutic benefit. In this circumstance, the EMA ensures that the evaluation for the opinion of the CHMP is completed within 150 days and the opinion issued thereafter.

For those medicinal products for which the centralized procedure is not available, the applicant must submit marketing authorization applications to the national medicines regulators through one of three procedures: (i) the mutual recognition procedure (which must be used if the product has already been authorized in at least one other E.U. member state, and in which the E.U. member states are required to grant an authorization recognizing the existing authorization in the other E.U. member state, unless they identify a serious risk to public health), (ii) the decentralized procedure (in which applications are submitted simultaneously in two or more E.U. member states) or (iii) national authorization procedures (which results in a marketing authorization in a single E.U. member state).

Mutual Recognition Procedure

The mutual recognition procedure, or MRP, for the approval of human drugs is an alternative approach to facilitate individual national marketing authorizations within the European Union. Basically, the MRP may be applied for all human drugs for which the centralized procedure is not obligatory. The MRP is applicable to the majority of conventional medicinal products and must be used if the product has already been authorized in one or more member states.

The characteristic of the MRP is that the procedure builds on an already–existing marketing authorization in a member state of the European Union that is used as a reference in order to obtain marketing authorizations in other E.U. member states. In the MRP, a marketing authorization for a drug already exists in one or more member states of the European Union and subsequently marketing authorization applications are made in other E.U. member states by referring to the initial marketing authorization. The member state in which the marketing authorization was first granted will then act as the reference member state. The member states where the marketing authorization is subsequently applied for act as concerned member states. The concerned member states are required to grant an authorization recognizing the existing authorization in the reference member state, unless they identify a serious risk to public health.

The MRP is based on the principle of the mutual recognition by E.U. member states of their respective national marketing authorizations. Based on a marketing authorization in the reference member state, the applicant may apply for marketing authorizations in other member states. In such case, the reference member state shall update its existing assessment report about the drug in 90 days. After the assessment is completed, copies of the report are sent to all member states, together with the approved summary of product characteristics, labeling and package leaflet. The concerned member states then have 90 days to

recognize the decision of the reference member state and the summary of product characteristics, labeling and package leaflet. National marketing authorizations shall be granted within 30 days after acknowledgement of the agreement.

If any E.U. member state refuses to recognize the marketing authorization by the reference member state, on the grounds of potential serious risk to public health, the issue will be referred to a coordination group. Within a timeframe of 60 days, member states shall, within the coordination group, make all efforts to reach a consensus. If this fails, the procedure is submitted to an EMA scientific committee for arbitration. The opinion of this EMA Committee is then forwarded to the European Commission for the start of the decision making process. As in the centralized procedure, this process entails consulting various European Commission Directorates General and the Standing Committee on Human Medicinal Products.

Data Exclusivity

In the European Union, marketing authorization applications for generic medicinal products do not need to include the results of pre-clinical and clinical trials, but instead can refer to the data included in the marketing authorization of a reference product for which regulatory data exclusivity has expired. If a marketing authorization is granted for a medicinal product containing a new active substance, that product benefits from eight years of data exclusivity, during which generic marketing authorization applications referring to the data of that product may not be accepted by the regulatory authorities, and a further two years of market exclusivity, during which such generic products may not be placed on the market. The two-year period may be extended to three years if during the first eight years a new therapeutic indication with significant clinical benefit over existing therapies is approved.

Orphan Medicinal Products

The EMA's Committee for Orphan Medicinal Products, or COMP, may recommend orphan medicinal product designation to promote the development of products that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than five in 10,000 persons in the European Union. Additionally, this designation is granted for products intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the product in the European Union would be sufficient to justify the necessary investment in developing the medicinal product. The COMP may only recommend orphan medicinal product designation when the product in question offers a significant clinical benefit over existing approved products for the relevant indication. Following a positive opinion by the COMP, the European Commission adopts a decision granting orphan status. The COMP will reassess orphan status in parallel with EMA review of a marketing authorization application and orphan status may be withdrawn at that stage if it no longer fulfills the orphan criteria (for instance because in the meantime a new product was approved for the indication and no convincing data are available to demonstrate a significant benefit over that product). Orphan medicinal product designation entitles a party to financial incentives such as a reduction of fees or fee waivers and 10 years of market exclusivity is granted following marketing authorization. During this period, the competent authorities may not accept or approve any similar medicinal product, unless it offers a significant clinical benefit. This period may be reduced to six years if the orphan medicinal product designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity.

Pediatric Development

In the European Union, companies developing a new medicinal product must agree to a Pediatric Investigation Plan, or PIP, with the EMA and must conduct pediatric clinical trials in accordance with that PIP unless a waiver applies, for example, because the relevant disease or condition occurs only in adults. The marketing authorization application for the product must include the results of pediatric clinical trials conducted in accordance with the PIP, unless a waiver applies, or a deferral has been granted, in which case the pediatric clinical trials must be completed at a later date. Products that are granted a marketing authorization on the basis of the pediatric clinical trials conducted in accordance with the PIP are eligible for a six-month extension of the protection under a supplementary protection certificate (if the product covered by it qualifies for one at the time of approval). This pediatric reward is subject to specific conditions and is not automatically available when data in compliance with the PIP are developed and submitted.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension of clinical trials, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Reimbursement

Sales of pharmaceutical products in the United States will depend, in part, on the extent to which the costs of the products will be covered by third-party payers, such as government health programs, and commercial insurance and managed health care organizations. These third-party payers are increasingly challenging the prices charged for medical products and services. Additionally, the containment of health care costs has become a priority of federal and state governments, and the prices of drugs have been a focus in this effort. The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs, including price controls, utilization management and requirements for substitution of generic products. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit our net revenue and results. If these third-party payers 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 on a profitable basis.

The Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or the MMA, imposed requirements for the distribution and pricing of prescription drugs for Medicare beneficiaries and included a major expansion of the prescription drug benefit under Medicare Part D. Under Part D, Medicare beneficiaries may enroll in prescription drug plans offered by private entities that provide coverage of outpatient prescription drugs. Part D is available through both stand-alone prescription drug benefit plans and prescription drug coverage as a supplement to Medicare Advantage plans. Unlike Medicare Parts A and B, Part D coverage is not standardized. Part D prescription drug plan sponsors are not required to pay for all covered Part D drugs, and each drug plan can develop its own drug formulary that identifies which drugs it will cover and at what tier or level. However, Part D prescription drug formularies must include drugs within each therapeutic category and class of covered Part D drugs, though not necessarily all the drugs in each category or class. Any formulary used by a Part D prescription drug plan must be developed and reviewed by a pharmacy and therapeutic committee.

Government payment for some of the costs of prescription drugs may increase demand for products for which we receive marketing approval. However, any negotiated prices for our products covered by a Part D prescription drug plan will likely be lower than the prices we might otherwise obtain. Moreover, while the MMA applies only to drug benefits for Medicare beneficiaries, private payers often follow Medicare coverage policy and payment limitations in setting their own payment rates. Any reduction in payment that results from the MMA may result in a similar reduction in payments from non-governmental payers.

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act of 2010, collectively, the ACA, was enacted with the goal of expanding coverage for the uninsured while at the same time containing overall health care costs. With regard to pharmaceutical products, among other things, the ACA expanded and increased industry rebates for drugs covered under Medicaid programs and made changes to the coverage requirements under the Medicare D program. There remain challenges to certain aspects of the ACA. Since January 2017, President Trump has signed two Executive Orders and other directives designed to eliminate the implementation of certain provisions of the ACA or otherwise circumvent some of the requirements for health insurance mandated by the ACA. Concurrently, Congress has considered legislation that would repeal or repeal and replace all or part of the ACA. While Congress has not passed comprehensive repeal legislation, several bills affecting the implementation of certain taxes under the ACA have been signed into law. The Tax Cuts and Jobs Act was enacted, which, among other things, included a provision which repealed, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the “individual mandate”. In addition, the 2020 federal spending package permanently eliminated, effective January 1, 2020, the ACA-mandated “Cadillac” tax on certain high-cost employer-sponsored health insurance plans and the medical device excise tax on non-exempt medical devices and, effective January 1, 2021, also eliminates the health insurer tax. On December 14, 2018, a Texas U.S. District Court Judge ruled that the ACA is unconstitutional in its entirety because the “individual mandate” was repealed by Congress as part of the Tax Cuts and Jobs Act. Additionally, on December 18, 2019, the U.S. Court of Appeals for the 5th Circuit upheld the District Court ruling that that the individual mandate was unconstitutional and remanded the case back to the District Court to determine whether the remaining provisions of the ACA are invalid as well. It is unclear how this decision, future decisions, subsequent appeals, and other efforts to repeal and replace the ACA will impact the ACA.

In addition, in some foreign countries, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country. For example, some E.U. jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical trials that compare the cost-effectiveness of a particular product candidate to currently available therapies. Other member states allow companies to fix their own prices for medicines but monitor and control company profits. Such differences in national pricing regimes may create price

differentials between E.U. member states. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products. Historically, products launched in the European Union do not follow price structures of the United States. In the European Union, the downward pressure on healthcare costs in general, particularly prescription medicines, has become intense. As a result, barriers to entry of new products are becoming increasingly high and patients are unlikely to use a drug product that is not reimbursed by their government.

Pharmaceutical Coverage, Pricing and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any drug products for which we obtain regulatory approval. In the United States, sales of any products for which we receive regulatory approval for commercial sale will depend in part on the availability of coverage and reimbursement from third-party payors. Third-party payors include government authorities, managed care providers, private health insurers and other organizations. Coverage and reimbursement policies for drug products can differ significantly from payor to payor as there is no uniform policy of coverage and reimbursement for drug products among third party payors in the United States. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement rates, but also have their own methods and approval process apart from Medicare determinations. The process for determining whether a payor will provide coverage for a drug product may be separate from the process for setting the reimbursement rate that the payor will pay for the drug product. Third-party payors may limit coverage to specific drug products on an approved list, or formulary, which might not include all of the FDA-approved drugs for a particular indication. Moreover, a payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

Third-party payors are increasingly challenging the price and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. In order to obtain 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 our products, in addition to the costs required to obtain regulatory approvals. Our product candidates may not be considered medically necessary or cost-effective. If third-party payors do not consider a product to be cost-effective compared to other available therapies, they may not cover the product after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow a company to sell its products at a profit.

Other Healthcare Laws and Compliance Requirements

Our current and future operations may subject us to various federal and state laws targeting fraud and abuse in the healthcare industry. These laws may impact, among other things, our research and proposed sales, marketing and education programs. In addition, we may be subject to patient privacy regulation by both the federal government and the states and foreign jurisdictions in which we conduct our business. The laws that may affect our ability to operate include:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, to induce, or in return for, the purchase or recommendation of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs;
- federal civil and criminal false claims laws and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid, or other third-party payers that are false or fraudulent;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal statutes that prohibit executing a scheme to defraud any healthcare benefit program and making false statements relating to healthcare matters;
- the federal transparency laws, including the federal Physician Payments Sunshine Act, that require drug manufacturers to disclose payments and other transfers of value provided to physicians, as defined by such law, and teaching hospitals;
- HIPAA, as amended by the Health Information Technology and Clinical Health Act, or HITECH, and its implementing regulations, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information; and
- Foreign and state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payer, including commercial insurers, state and local laws governing the disclosure of payments to health care professionals, state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government, state laws that require the reporting of information related to drug pricing, state and local laws requiring the registration of pharmaceutical sales representatives and state laws governing the privacy and

security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

- If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to it, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion of products from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations.

Employees

As of December 31, 2019, we employed 58 full-time employees. Of these employees, 38 were engaged in research and development and 20 were engaged in general and administrative functions. All of our employees are located in the United States and Vienna, Austria. We have no collective bargaining agreements with our employees and have not experienced any work stoppages. We consider our relationship with our employees to be good.

Corporate Information

We were incorporated under the laws of the State of Delaware in 2010 under the name Arsanis Inc. Following the Merger on March 13, 2019 with X4 Therapeutics Inc. (formerly X4 Pharmaceuticals Inc.), we changed our name to X4 Pharmaceuticals, Inc. Our principal executive offices are located at 955 Massachusetts Avenue, 4th Floor, Cambridge, Massachusetts 02139 and our telephone number is (857) 529-8300.

Available Information

We maintain a website at http://www.x4pharma.com. Our annual reports on Form 10-K, quarterly reports on Form 10-Q, proxy statements, current reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Exchange Act are available free of charge on our website as soon as reasonably practicable after electronically filing such reports with the SEC. Such reports and other information may be accessed through the SEC's website at www.sec.gov. Information contained in our website is not part of this or any other report that we file with or furnish to the SEC.

ITEM 1A. RISK FACTORS

An investment in shares of our common stock involves a high degree of risk. You should carefully consider the following information about these risks, together with the other information appearing in our Annual Report on Form 10-K for the year ended December 31, 2019, before deciding to invest in our common stock. The occurrence of any of the following risks could have a material adverse effect on our business, financial condition, results of operations and future growth prospects. In these circumstances, the market price of our common stock could decline, and you may lose all or part of your investment.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred significant losses since our inception. We expect to continue to incur losses and may never generate profits from operations or maintain profitability.

Since inception, we have incurred significant operating losses. Without regard to the historical operating results of our predecessor, Arsanis, our net losses were $52.8 million, $33.3 million and $22.0 million for the years ended December 31, 2019, 2018 and 2017 respectively, and we had an accumulated deficit of $132.0 million as of December 31, 2019. To date, we have financed our operations primarily through issuances of shares of common stock warrants and prefunded warrants for the purchase of our preferred stock and our common stock, sales of preferred stock, proceeds from the issuance of convertible debt and borrowings under loan and security agreements. In 2019, we completed a merger with Arsanis and acquired its $26.4 million of cash, cash equivalents and restricted cash. We have not generated any revenue from product sales to date. We have devoted substantially all of our efforts to research and development, including clinical trials. We have not completed the development of any drugs. We expect to continue to incur significant expenses and increasing operating losses for at least the next few years as we conduct additional clinical trials for our product candidates; continue to discover and develop additional product candidates; acquire or in-license other product candidates and technologies; maintain, expand and protect our intellectual property portfolio; hire additional clinical, scientific and commercial personnel; establish a commercial manufacturing source and secure supply chain capacity sufficient to provide commercial quantities of any product candidates for which we may obtain regulatory approval; seek regulatory approvals for any product candidates that successfully complete clinical trials; establish a sales,

marketing and distribution infrastructure to commercialize any products for which we may obtain regulatory approval; and add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenues. Even if we achieve profitability in the future, we may not be able to sustain profitability in subsequent periods. The net losses we incur may fluctuate significantly from quarter to quarter and year to year.

Our ability to generate profits from operations and thereafter to remain profitable depends heavily on:

- the scope, number, progress, duration, endpoints, cost, results and timing of clinical trials and nonclinical studies of our current or potential future product candidates, including in particular the scope, progress, duration, endpoints, cost, results and timing for completion of our Phase 3 trial of mavorixafor for the treatment of WHIM syndrome, our Phase 1b clinical trial of mavorixafor for the treatment of severe congenital neutropenia, or SCN, and our Phase 1b clinical trial of mavorixafor for the treatment of Waldenström macroglobulinemia.
- our ability to raise sufficient funds to support the development and potential commercialization of our product candidates;
- the outcomes and timing of regulatory reviews, approvals or other actions;
- our ability to obtain marketing approval for our product candidates;
- our ability to establish and maintain licensing, collaboration or similar arrangements on favorable terms and whether and to what extent we retain development or commercialization responsibilities under any new licensing, collaboration or similar arrangement;
- the success of any other business, product or technology that we acquire or in which we invest;
- our ability to maintain, expand and defend the scope of our intellectual property portfolio;
- our ability to manufacture any approved products on commercially reasonable terms;
- our ability to establish a sales and marketing organization or suitable third-party alternatives for any approved product; and
- the number and characteristics of product candidates and programs that we pursue.

Based on our current plans, which will allow us to fund our operations for at least the 12 months following the filing of this Annual Report or Form 10-K, we do not expect to generate significant revenue from product sales unless and until we (or a potential future licensee or collaborator) obtain marketing approval for, and commercialize, one or more of our current or potential future product candidates. Neither we nor a licensee may ever succeed in obtaining marketing approval for, or commercializing, our product candidates and, even if we do, we may never generate revenues that are significant enough to generate profits from operations. Even if we do generate profits from operations, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to generate profits from operations and remain profitable would decrease our value and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our product offerings or continue our operations. A decline in our value could also cause you to lose all or part of your investment.

Our limited operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

Without regard to the historical operations of Arsanis, our operations to date have been limited to organizing the company, entering into licensing arrangements for mavorixafor, hiring a team of experienced personnel, raising capital, and undertaking nonclinical studies and clinical trials and regulatory activities for our development programs, primarily mavorixafor. We have not yet demonstrated our ability to successfully complete development of any product candidate, including large-scale, pivotal clinical trials required for regulatory approval of our product candidates, obtain marketing approvals, manufacture a commercial scale product, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization.

Typically, it takes many years to develop one new product from the time it is discovered to when it is commercially available, if ever. In addition, the CXCR4 receptor that we are pursuing with respect to our product candidates is a relatively novel target with a limited research and development history. Consequently, any early predictions made about our future success or viability may not be as accurate as they could be if we had a longer operating history.

As an early-stage company, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors that may alter or delay our plans. Assuming that we complete the development of and obtain marketing approval for any of our product candidates, we will need to transition from a company with a research and development focus to a company capable of supporting commercial activities. We may encounter unforeseen expenses, difficulties, complications and delays, and may not be successful in such a transition.

We will require substantial additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate any product development programs or commercialization efforts.

Our operations have consumed a large amount of cash since inception. We expect our research and development expenses to increase in future periods as we continue to advance the clinical development of our product candidates and prepare for the launch and commercialization of any product candidates for which we receive regulatory approval, including potentially building our own commercial organization to address the United States and certain other markets. In addition, if we obtain marketing approval for any of our product candidates that are not then subject to licensing, collaboration or similar arrangements with third parties, we expect to incur significant commercialization expenses related to product sales, marketing, distribution and manufacturing. Furthermore, we expect to incur additional costs associated with operating as a public company in the United States. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations.

We cannot be certain that additional funding will be available on acceptable terms, or at all. If we are unable to raise additional capital when needed or in sufficient amounts or on terms acceptable to it, we could be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts of one or more of our product candidates or one or more of our other research and development initiatives. We also could be required to:

- seek collaborators for one or more of our current or future product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available; or
- relinquish or license on unfavorable terms our rights to technologies or product candidates that we otherwise would seek to develop or commercialize ourselves.

Our future funding requirements, both near and long-term, will depend on many factors, including, but not limited to:

- the scope, number, initiation, progress, timing, costs, design, duration, any potential delays, and results of clinical trials and nonclinical studies for our current or future product candidates, particularly our Phase 3 trial of mavorixafor for the treatment of WHIM syndrome, our Phase 1b clinical trial of mavorixafor for the treatment of SCN, and our Phase 1b clinical trial of mavorixafor for the treatment of WM;
- the clinical development plans that we establish for these product candidates;
- the number and characteristics of product candidates and programs that we develop or may in-license;
- the outcome, timing and cost of regulatory reviews, approvals or other actions to meet regulatory requirements established by the U.S. Food and Drug Administration, or FDA, and comparable foreign regulatory authorities, including the potential for the FDA or comparable foreign regulatory authorities to require that we perform more studies for our product candidates than those that we currently expect;
- our ability to obtain marketing approval for our product candidates;
- the cost of filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights covering our product candidates, including any such patent claims and intellectual property rights that we have licensed from Genzyme pursuant to the terms of our license agreement with Genzyme or from other third parties;
- our ability to maintain, expand and defend the scope of our intellectual property portfolio, including the cost of defending intellectual property disputes, including patent infringement actions brought by third parties against us or our product candidates;

- the cost and timing of completion of commercial-scale outsourced manufacturing activities with respect to our product candidates;
- our ability to establish and maintain licensing, collaboration or similar arrangements on favorable terms and whether and to what extent we retain development or commercialization responsibilities under any new licensing, collaboration or similar arrangement;
- the cost of establishing sales, marketing and distribution capabilities for any product candidates for which we may receive regulatory approval in regions where we choose to commercialize our products on our own;
- the success of any other business, product or technology that we acquire or in which we invest;
- the costs of acquiring, licensing or investing in businesses, product candidates and technologies;
- our need and ability to hire additional management and scientific and medical personnel;
- the costs to operate as a public company, including the need to implement additional financial and reporting systems and other internal systems and infrastructure for our business;
- market acceptance of our product candidates, to the extent any are approved for commercial sale; and
- the effect of competing technological and market developments.

Conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of products that will not be commercially available for sale by us for at least the next few years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. In addition, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans. Additional financing may not be available to us on acceptable terms, or at all. The unavailability of additional financing on acceptable terms, or at all, would have an adverse effect on your investment.

Raising additional capital may cause dilution to our investors, restrict our operations or require us to relinquish rights to our technologies or product candidates. Future debt obligations may expose us to risks that could adversely affect our business, operating results and financial condition and may result in further dilution to our stockholders.

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, and licensing, collaboration or similar arrangements. We do not have any committed external sources of funds and may seek to raise additional capital at any time. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a holder of our common stock. 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, declaring dividends or other distributions, acquiring or licensing intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business and may result in liens being placed on our assets and intellectual property. If we default on such indebtedness, we could lose such assets and intellectual property.

If we raise additional funds through licensing, collaboration or similar arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research and development programs or product candidates or grant licenses on terms that are not favorable to us. If we are unable to raise additional funds through equity or debt financings or through licensing, collaboration or similar arrangements 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.

We have not generated any revenues since inception and may never become profitable.

To date, we have not generated any revenues. Our ability to generate revenue and become profitable depends upon our ability to successfully obtain marketing approval and commercialize our product candidates, including mavorixafor-based product candidates, X4P-002, X4P-003 or other product candidates that we may develop, in-license or acquire in the future. Even if we are able to successfully achieve regulatory approval for these product candidates, we are unable to predict the extent of any future

losses and do not know when any of these product candidates will generate revenue for us, if at all. Our ability to generate revenue from mavorixafor or other product candidates also depends on a number of additional factors, including our ability to:

- successfully complete development activities, including all necessary nonclinical studies and clinical trials;
- complete and submit New Drug Applications, or NDAs, to the FDA and obtain regulatory approval for indications for which there is a commercial market;
- complete and submit marketing applications to, and obtain regulatory approval from, foreign regulatory authorities;
- set and obtain a commercially viable price for our products;
- obtain commercial quantities of our products at acceptable cost levels;
- develop a commercial organization capable of sales, marketing and distribution for the products we intend to sell ourselves in the markets in which we have retained commercialization rights;
- find suitable collaborators to help us market, sell and distribute our approved products in other markets; and
- obtain coverage and adequate reimbursement from third-party, including government, payors.

In addition, because of the numerous risks and uncertainties associated with product development, including the possibility that our product candidates may not advance through development or demonstrate safety and efficacy for their intended uses, the FDA or any other regulatory agency may require additional clinical trials or nonclinical studies. We are unable to predict the timing or amount of increased expenses, or when or if we will be able to achieve or maintain profitability, and such expense could increase beyond our expectations if the FDA or any other regulatory agency requires such additional clinical trials or nonclinical studies as part of the application and approval process or post-approval process if we are successful at achieving regulatory approval. Even if we are able to successfully complete the development and regulatory reviews described above, we anticipate incurring significant costs associated with commercializing these products, if they are approved.

Even if we are able to generate revenues from the sale of our product candidates, we may not become profitable and may need to obtain additional funding to continue operations. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce our operations. 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 discovery and preclinical development efforts, expand our business or continue our operations and may require us to raise additional capital that may dilute your ownership interest. A decline in our value could also cause you to lose all or part of your investment.

Risks Related to Development and Commercialization of Our Product Candidates

We depend almost entirely on the success of our lead product candidate, mavorixafor, which we are developing initially for the treatment of WHIM syndrome, for the treatment of SCN, for the treatment of WM, and with a potential strategic partner, for the treatment of ccRCC. We cannot be certain that we will be able to obtain regulatory approval for, or successfully commercialize, mavorixafor or any other product candidate.

Our business depends almost entirely on the successful clinical development, regulatory approval and commercialization of mavorixafor. We currently have no drug product for sale and may never be able to develop marketable drug products. We initiated a global Phase 3 pivotal clinical trial of our lead product candidate, mavorixafor, in WHIM patients in the second quarter of 2019, and may be required to complete additional nonclinical studies and clinical trials before we can seek regulatory approval. In the fourth quarter of 2019, we also initiated a Phase 1b clinical trial of mavorixafor for the treatment of SCN and WM. Having completed our Phase 2a clinical trial, we do not plan to develop mavorixafor for the treatment of ccRCC on our own. Our other programs, including X4P-002 and X4P-003, are still in the preclinical development stage. The clinical trials of our product candidates are, and the manufacturing and marketing of our product candidates will be, subject to extensive and rigorous review and regulation by government authorities in the United States and in other countries where we intend to test and, if approved, market any product candidate. Before obtaining regulatory approvals for the commercial sale of any product candidate, we must successfully meet a number of critical developmental milestones, including:

- developing dosages that will be well-tolerated, safe and effective;

- completing the development and scale-up to permit manufacture of our product candidates in commercial quantities and at acceptable costs;
- demonstrating through pivotal clinical trials that each product candidate is safe and effective in patients for the intended indication;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers; and
- obtaining and maintaining patent and trade secret protection and non-patent exclusivity for our product candidates.

The time necessary to achieve these developmental milestones for any individual product candidate is long and uncertain, and we may not successfully complete these milestones for mavorixafor or any other product candidates that we may develop. We have not yet completed development of any product candidate. We also may not be able to finalize the design or formulation for our other programs, X4P-002 for the treatment of glioblastoma multiforme, or GBM, and X4P-003, a next generation molecule for the treatment of rare diseases linked to defects in CXCR4 trafficking.

We are continuing to test and develop our product candidates and may explore possible design or formulation changes to address safety, efficacy, manufacturing efficiency and performance issues to the extent any arise. We may not be able to complete development of any product candidates that demonstrate safety and efficacy and that will have a commercially reasonable treatment and storage period. If we are unable to complete development of mavorixafor or any other product candidates that we may develop, we will not be able to commercialize and earn revenue from them.

We expect to develop mavorixafor, and potentially future product candidates, in combination with other therapies, which exposes us to additional risks.

We intend to develop mavorixafor, and may develop future product candidates, in combination with one or more currently approved cancer therapies. Even if any product candidate we develop were to receive marketing approval or be commercialized for use in combination with other existing therapies, we would continue to be subject to the risks that the FDA or similar regulatory authorities outside of the United States could revoke approval of the therapy used in combination with our product candidate or that safety, efficacy, manufacturing or supply issues could arise with these existing therapies. Combination therapies are commonly used for the treatment of cancer, and we would be subject to similar risks if we develop any of our product candidates for use in combination with other drugs or for indications other than cancer. This could result in our own products being removed from the market or being less successful commercially.

We may also evaluate mavorixafor or any other future product candidates in combination with one or more other cancer therapies that have not yet been approved for marketing by the FDA or similar regulatory authorities outside of the United States. We will not be able to market and sell mavorixafor or any product candidate we develop in combination with any such unapproved cancer therapies that do not ultimately obtain marketing approval.

If the FDA or similar regulatory authorities outside of the United States do not approve these other drugs or revoke their approval of, or if safety, efficacy, manufacturing or supply issues arise with, the drugs that we choose to evaluate in combination with mavorixafor or any product candidate we develop, we may be unable to obtain approval of or market mavorixafor or any product candidate we develop.

The regulatory review and approval processes of the FDA and comparable foreign regulatory authorities are lengthy, time-consuming and inherently unpredictable, and if we are ultimately unable to obtain regulatory approval for our product candidates, including mavorixafor, our business will be substantially harmed.

Of the large number of drugs in development in the United States, only a small percentage receive FDA regulatory approval and are commercialized in the United States. We are not permitted to market mavorixafor or any other product candidate in the United States until we receive approval of an NDA from the FDA, or in any foreign countries until we receive the requisite approval from such countries or jurisdictions, such as the marketing authorization application, or MAA, in the European Union from the European Medicines Agency, or EMA. Prior to submitting an NDA to the FDA for approval of mavorixafor for the treatment of WHIM syndrome, we will need to successfully complete the current Phase 3 pivotal clinical trial of mavorixafor in patients with WHIM syndrome and potentially additional clinical trials and/or nonclinical studies. Successfully completing clinical trials and

obtaining approval of an NDA is a complex, lengthy, expensive and uncertain process, and the FDA, or a comparable foreign regulatory authority, may delay, limit or deny approval of mavorixafor for the treatment of WHIM syndrome or other indications for many reasons, including, among others:

- disagreement with the design or implementation of our clinical trials;
- disagreement with the sufficiency of our clinical trials;
- failure to demonstrate the safety and efficacy of mavorixafor or any other product candidate for its proposed indications;
- failure to demonstrate that any clinical and other benefits of mavorixafor or any other product candidate outweigh its safety risks;
- a negative interpretation of the data from our nonclinical studies or clinical trials;
- deficiencies in the manufacturing or control processes or failure of third-party manufacturing facilities with which we contract for clinical and commercial supplies to comply with current Good Manufacturing Practice requirements, or cGMPs;
- insufficient data collected from clinical trials of mavorixafor or changes in the approval requirements that render its nonclinical and clinical data insufficient to support the filing of an NDA or to obtain regulatory approval; or
- changes in clinical practice in or approved products available for the treatment of the target patient population that could have an impact on the indications that we are pursuing for mavorixafor or our other product candidates.

The FDA or a comparable foreign regulatory authority may also require more information, including additional nonclinical or clinical data to support approval, which may delay or prevent approval of our commercialization plans, or cause us to abandon the development program. Even if we obtain regulatory approval, our product candidates may be approved for fewer or more limited indications than we request, such approval may be contingent on the performance of costly post-marketing clinical trials, or we may not be allowed to include the labeling claims necessary or desirable for the successful commercialization of such product candidate. For instance, it is possible that mavorixafor could be approved for an indication but fail to be used for treating patients in that indication due to the availability of other available treatments or then-accepted clinical practice.

We depend on license agreements with Genzyme, Beth Israel Deaconess Medical Center and Georgetown University to permit us to use patents and patent applications. Termination of these rights or the failure to comply with obligations under these agreements could materially harm our business and prevent us from developing or commercializing our product candidates.

We are party to license agreements with Genzyme, Beth Israel Deaconess Medical Center and Georgetown University under which we were granted rights to patents and patent applications that are important to our business. We rely on these license agreements in order to be able to use various proprietary technologies that are material to our business, including certain patents and patent applications that cover our product candidates, including mavorixafor. Our rights to use these patents and patent applications and employ the inventions claimed in these licensed patents are subject to the continuation of and our compliance with the terms of our license agreements.

Our license agreement with Genzyme imposes upon us various diligence, payment and other obligations, including the following:

- our obligation to pay Genzyme milestone payments in the aggregate amount of up to $25.0 million, contingent upon our achievement of certain late-stage regulatory and sales milestones with respect to licensed products.
- our obligation to pay Genzyme tiered royalties based on net sales of licensed products that we commercialize under the agreement.
- our obligation to pay Genzyme a certain percentage of cash payments received by us or our affiliates in consideration for the grant of a sublicense under the license granted to us by Genzyme.

If we fail to comply with any of our obligations under the Genzyme license agreement, or we are subject to a bankruptcy, Genzyme may have the right to terminate the license agreement, in which event we would not be able to market any product candidates covered by the license.

Prior to July 2014, we did not control the prosecution, maintenance, or filing of the patents and patent applications that are licensed to us under the Genzyme license agreement, or the enforcement of these patents and patent applications against infringement by third parties. Thus, these patents and patent applications were not drafted by us or our attorneys, and we did not control or have any input into the prosecution of these patents and patent applications prior to our execution of the Genzyme license agreement in July 2014. Under the terms of the license agreement with Genzyme, since July 2014, we have controlled the right to control the prosecution, maintenance, and filing of the patents and patent applications that are licensed to us, and the enforcement of these patents and patent applications against infringement by third parties. However, we cannot be certain that the same level of attention was given to the drafting and prosecution of these patents and patent applications as we may have used if we had control over the drafting and prosecution of such patents and patent applications. We also cannot be certain that drafting or prosecution of the patents and patent applications licensed to us has been conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents.

Pursuant to our license agreement with Beth Israel Deaconess Medical Center, we paid an upfront, one-time fee for the rights granted by the license agreement. This license agreement imposes upon us various obligations, including the requirement to provide Beth Israel Deaconess Medical Center with progress reports at regular intervals and to maintain specified levels of insurance. Beth Israel Deaconess Medical Center may terminate the agreement for our non-payment, insolvency or default of material obligations. We have the right to terminate the agreement for any reason upon 90 days' advance written notice.

Our license agreement with Georgetown imposes upon us various diligence, payment and other obligations, including our obligations to pay Georgetown milestone payments in the aggregate amount of up to $0.8 million, contingent upon our achievement of certain sales milestones with respect to licensed products, to deliver reports upon certain events and at regular intervals and to maintain customary levels of insurance. Georgetown may terminate the agreement for our non-payment, insolvency, failure to maintain insurance or default of material obligations. We have the right to terminate the agreement for any reason upon 60 days advance written notice.

Disputes may arise under any of our license agreements with Genzyme, Beth Israel Deaconess Medical Center and/or Georgetown University regarding the intellectual property that is subject to such license agreement, including:

- the scope of rights granted under the applicable license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property that is not subject to the applicable license agreement;
- our diligence obligations with respect to the use of the licensed technology under the applicable license agreement to develop and commercialize products and technologies, including the level of effort and specific activities that will satisfy those diligence obligations; and
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by us and our collaborators.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain any of our license agreements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates and technologies.

The results of clinical trials may not support our product candidate claims.

Even if our clinical trials are completed as planned, we cannot be certain that their results will support the proposed product candidates, that the FDA or foreign government authorities will agree with our conclusions regarding such results, or that the FDA or foreign governmental authorities will not require additional clinical trials. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful and the results of later clinical trials often do not replicate the results of prior clinical trials and preclinical testing. The clinical trial results may fail to demonstrate that our product candidates are safe for humans and effective for the intended indications. This failure could cause us to abandon a product candidate and may delay development of other product candidates. Any delay in, or termination of, our clinical trials will delay or prevent the submission of our marketing applications (NDA and/or MAA) and, ultimately, our ability to obtain approval and commercialize

our product candidates and generate product revenues. Information about certain clinical trials, including results (positive or negative) will be made public according to each country's clinical trial register policies. Competitors may use this publicly available information to gain knowledge regarding the progress of development programs.

Delays in our clinical trials may lead to a delay in the submission of our marketing approval application and jeopardize our ability to potentially receive approvals and generate revenues from the sale of our products.

We may experience delays in our current or future clinical trials, including our Phase 3 trial of mavorixafor for the treatment of WHIM syndrome, our Phase 1b clinical trial of mavorixafor for the treatment of SCN, and our Phase 1b clinical trial of mavorixafor for the treatment of WM. We do not know whether planned clinical trials will begin or enroll subjects on time, need to be redesigned or be completed on schedule, if at all. Clinical trials may be delayed, suspended or terminated for a variety of reasons, such as:

- delay or failure in reaching agreement with the FDA or a comparable foreign regulatory authority on a trial design that we are able to execute;
- delay or failure in obtaining authorization to commence a trial or inability to comply with conditions imposed by a regulatory authority regarding the scope or design of a clinical trial;
- inability, delay or failure in identifying and maintaining a sufficient number of trial sites, many of which may already be engaged in competing clinical trial programs;
- delay or failure in recruiting and enrolling suitable subjects to participate in a trial;
- delay or failure in having subjects complete a trial or return for post-treatment follow-up;
- clinical sites and investigators deviating from trial protocol, failing to conduct the trial in accordance with regulatory requirements, or dropping out of a trial;
- delay or failure in reaching agreement on acceptable terms with prospective clinical research organizations, or CROs, and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- delay or failure in obtaining institutional review board, or IRB, approval to conduct a clinical trial at each site;
- delays resulting from negative or equivocal findings of the Data Safety Monitoring Board, or DSMB, if any;
- ambiguous or negative results;
- decision by the FDA, a comparable foreign regulatory authority, or recommendation by a DSMB to suspend or terminate clinical trials at any time for safety issues or for any other reason;
- inadequate drug product for use in nonclinical studies or clinical trials;
- lack of adequate funding to continue the product development program; or
- changes in governmental regulations or requirements.

Any delays in completing our clinical trials will increase our costs, slow down our product candidate development and approval process and jeopardize our ability to commence product sales and generate revenues. Any of these occurrences may significantly harm our business, financial condition and prospects. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of our product candidates.

We may fail to enroll a sufficient number of patients in our clinical trials in a timely manner, which could delay or prevent clinical trials of our product candidates.

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. The timing of our clinical trials depends on the rate at which we can recruit and enroll patients in testing our product candidates. The timing of our clinical trials depends in part on the speed at which we can recruit patients to participate in testing mavorixafor and any other current or future product candidates that we may develop as well as completion of required follow-up periods. If we cannot identify patients to participate in our clinical trials or if patients are unwilling to participate in our clinical trials for any reason,

including if patients choose to enroll in competitive clinical trials for similar patient populations, the timeline for recruiting patients, conducting studies and obtaining regulatory approval of mavorixafor and any other current or future product candidates that we may develop may be delayed. These delays could result in increased costs, delays in advancing our current or future product candidates, including mavorixafor, X4P-002 or X4P-003, delays in testing the effectiveness of our product candidates or termination of the clinical trials altogether.

We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics to achieve diversity in a trial, to complete our clinical trials in a timely manner. In particular, we are currently evaluating mavorixafor for the treatment of WHIM syndrome, SCN and WM, rare diseases with limited patient pools from which to draw for clinical trials. The eligibility criteria of our clinical trials will further limit the pool of available trial participants.

Patient enrollment, a significant factor in the duration of clinical trials, is also affected by many factors, including:

- the severity of the disease under investigation;
- the size and nature of the patient population (particularly with respect to orphan drugs which, by definition, are intended for a relatively small patient population);
- the eligibility criteria for the clinical trial in question;
- the design of the clinical trial;
- the inability to obtain and maintain patient consents;
- the risk that enrolled subjects will drop out before completion;
- clinicians' and patients' perceptions as to the potential advantages of the drug being studied in relation to other available therapies, including any new drug that may be approved or for which clinical trials are initiated for the indications that we are investigating;
- our CROs and our trial sites' efforts to facilitate timely screening and enrollment in clinical trials;
- patient referral practices of physicians; and
- our ability to monitor patients adequately during and after treatment.

We have made certain assumptions about the rate at which we can enroll patients in our clinical trials. To the extent that we do not meet this enrollment target, our projected timeline for development of our product candidates may be slowed. We rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials and while we will have agreements governing their activities, we have limited control over their actual performance.

If we experience difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may be forced to delay, limit or terminate ongoing or planned clinical trials of our product candidates, which would delay our ability to obtain approvals and generate product revenues from any of these product candidates.

If the commercial opportunity in WHIM syndrome, SCN or WM is smaller than we anticipate, our potential future revenue from mavorixafor for the treatment of any of the diseases may be adversely affected and our business may suffer.

If the size of the commercial opportunities in any of our target indications is smaller than we anticipate, we may not be able to achieve profitability and growth. We are developing mavorixafor initially as a treatment for patients with WHIM syndrome and also as a treatment for other rare diseases, including primary immunodeficiencies such as SCN and cancer such as WM. WHIM syndrome, SCN and WM each have a limited patient population.

For example, we are aware of only a few small available patient registries for WHIM syndrome, and we rely on various estimates and assumptions to estimate the addressable WHIM syndrome population. Based on a preliminary independent market research study conducted by a third-party research firm study that we sponsored, we estimate there are more than 1,000 genetically confirmed WHIM patients in the United States. If the commercial opportunity in WHIM syndrome is smaller than we anticipate, whether because our estimates of the addressable patient population prove to be incorrect or for other reasons, our potential future revenue from mavorixafor may be adversely affected and our business may suffer.

It is critical to our ability to grow and become profitable that we successfully identify patients with WHIM syndrome, SCN and WM. Our projections of the number of people who have WHIM syndrome (or its other potential primary immunodeficiencies), SCN or WM are based on a variety of sources, including third-party estimates and analyses in the scientific literature, and may prove to be incorrect. Further, new information may emerge that changes our estimate of the prevalence of these diseases or the number of patient candidates for each disease. The effort to identify patients for treatment is at an early stage, and we cannot accurately predict the number of patients for whom treatment might be possible. Additionally, the addressable patient population for our indications may be limited or may not be amenable to treatment with mavorixafor, and new patients may become increasingly difficult to identify or gain access to, which would adversely affect our results of operations and our business.

If we experience any of a number of possible unforeseen events in connection with our clinical trials, potential marketing approval or commercialization of our product candidates, or our entry into licensing, collaboration or similar arrangements, could be delayed or prevented.

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:

- clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development programs;
- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials at a higher rate than we anticipate;
- we may be unable to recruit and enroll a sufficient number of patients in our clinical trials to ensure adequate statistical power to detect any statistically significant treatment effects;
- 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, institutional review boards or independent ethics committees may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we may experience delays in reaching, or we may fail to reach, agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;
- we may have to suspend or terminate clinical trials of our product candidates for various reasons, including a finding that the participants are being exposed to unacceptable health risks or undesirable side effects;
- regulators, institutional review boards or independent ethics committees may require that we or our investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- the cost of clinical trials of our product candidates may be greater than we anticipate;
- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate; and
- our product candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators, institutional review boards or independent ethics committees to suspend or terminate the clinical trials.

Our product development costs will increase if we experience delays in testing or marketing approvals. We do not know whether any preclinical tests or clinical trials will begin as planned, will need to be redesigned or will be completed on schedule, or at all. Significant preclinical study or clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates, if they are approved, or allow our competitors to bring products to market before we do and impair our ability to successfully commercialize our product candidates, which may harm our business and results of operations.

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 specific product candidates. Currently, we are focusing our resources predominantly on the development mavorixafor for the treatment of WHIM syndrome, for the treatment of SCN and for the treatment of WM. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that have or that could later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or alternate and/or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

Risks Related to the Marketing and Commercialization of Our Product Candidates

If we are unable to establish sales and marketing capabilities to market and sell our product candidates, we may be unable to generate any revenue.

Even if we are ultimately successful in obtaining regulatory approval of mavorixafor for the treatment of WHIM syndrome or another indication, in order to market and sell mavorixafor and our other product candidates in development, we currently intend to build and develop our own sales, marketing and distribution operations. Although our management team has previous experience with such efforts, there can be no assurance that we will be successful in building these operations. If we are unable to establish adequate sales, marketing and distribution capabilities, we may not be able to generate product revenue and may not become profitable. We will also be competing with many companies that currently have extensive and well-funded sales and marketing operations. If any of our product candidates are approved, we may be unable to compete successfully against these more established companies.

Our commercial success depends upon attaining significant market acceptance of our product candidates, if approved, among hospitals, physicians, patients and healthcare payors.

Even if we obtain regulatory approval for any of our product candidates that we may develop or acquire in the future, the product may not gain market acceptance among hospitals, physicians, health care payors, patients and the medical community. Market acceptance of any of our product candidates for which we receive approval depends on a number of factors, including:

- the efficacy and safety of such product candidates as demonstrated in clinical trials;
- the clinical indications for which the product candidate is approved;
- acceptance by major operators of hospitals, physicians and patients of the product candidate as a safe and effective treatment, particularly the ability of mavorixafor and our other product candidates to establish themselves as a new standard of care in the treatment paradigm for the indications that we are pursuing;
- the potential and perceived advantages of our product candidates over alternative treatments as compared to the relative costs of the product candidates and alternative treatments;
- the prevalence and severity of any side effects with respect to our product candidates, including mavorixafor;
- our ability to offer any approved products for sale at competitive prices;
- the timing of market introduction of our products as well as competitive products;
- our pricing, and the availability of coverage and adequate reimbursement by third party payors and government authorities;
- relative convenience and ease of administration; and
- the effectiveness of our sales and marketing efforts and those of our potential future collaborators.

There may be delays in getting our product candidates, if approved, on hospital or insurance formularies or limitations on coverages that may be available in the early stages of commercialization for newly approved drugs. If any of our product candidates are approved but fail to achieve market acceptance among hospitals, physicians, patients or health care payors, we will

not be able to generate significant revenues, which would have a material adverse effect on our business, prospects, financial condition and results of operations.

Product candidates may cause undesirable side effects that could delay or prevent their marketing approval, limit the commercial profile of an approved label, or result in significant negative consequences following marketing approval, if any, including marketing withdrawal.

Undesirable side effects caused by any of our product candidates that we may develop or acquire could cause us or the FDA or other regulatory authorities to interrupt, delay or halt our clinical trials and could result in more restrictive labels or the delay or denial of marketing approval by the FDA or other regulatory authorities of such product candidates. Results of our clinical trials could reveal a high and unacceptable severity and prevalence of these or other 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 product candidates for any or all targeted indications. In addition, any drug-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims. Any of these occurrences may harm our business, financial condition and prospects significantly.

Further, clinical trials by their nature utilize a sample of the potential patient population. With a limited number of patients, rare and severe side effects of our product candidates may only be uncovered with a significantly larger number of patients exposed to the product candidate. If our product candidates receive marketing approval and we or others identify undesirable side effects caused by such product candidates (or any other similar drugs) after such approval, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw or limit their approval of such product candidates;
- regulatory authorities may require the addition of labeling statements, such as a “boxed” warning or a contraindication;
- we may be required to create a medication guide outlining the risks of such side effects for distribution to patients;
- we may be required to change the way such product candidates are distributed or administered, conduct additional clinical trials or change the labeling of the product candidates;
- regulatory authorities may require a Risk Evaluation and Mitigation Strategy plan to mitigate risks, which could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools;
- we may be subject to regulatory investigations and government enforcement actions;
- we may decide to remove such product candidates from the marketplace after they are approved;
- we could be sued and held liable for injury caused to individuals exposed to or taking our product candidates; and
- our reputation may suffer.

We believe that any of these events could prevent us from achieving or maintaining market acceptance of the affected product candidates and could substantially increase the costs of commercializing our product candidates, if approved, and significantly impact our ability to successfully commercialize our product candidates and generate revenues.

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

Although we do not currently have any drugs on the market, we are, and once we begin commercializing our product candidates, we will be subject to additional healthcare statutory and regulatory requirements and enforcement by the federal government and the states and foreign governments in the jurisdictions in which we conduct our business. Healthcare providers, physicians and third-party payors will play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our current and future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements

and relationships through which we market, sell and distribute any products for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations include the following:

- the federal Anti-Kickback Statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or paying remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, 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 a federal healthcare program such as Medicare and Medicaid; a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- the federal false claims laws impose criminal and civil penalties, including civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government; 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 False Claims Act;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program, or 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; similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- the federal physician payment transparency requirements, sometimes referred to as the “Sunshine Act” under the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively the ACA, require manufacturers of drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid, or the Children’s Health Insurance Program to report to CMS information related to payments and other transfers of value to physicians, as defined by such law, and teaching hospitals and the ownership and investment interests of physicians and their immediate family members in such manufacturers;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, which also imposes obligations on certain covered entity healthcare providers, health plans, and healthcare clearinghouses as well as their business associates that perform certain services involving the use or disclosure of individually identifiable health information, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- analogous state and foreign 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;
- some state laws require pharmaceutical companies to comply with the pharmaceutical industry’s voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and may require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures;
- state and local laws that require the registration of pharmaceutical sales representatives; and
- state and foreign laws also govern the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices 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 it, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion of products from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of

our operations. If any of the physicians or other healthcare 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 significant criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

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

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

In the United States, Medicare covers certain drug purchases by the elderly and eligible disabled people and introduced a reimbursement methodology based on average sales prices for physician-administered drugs. In addition, Medicare may limit the number of drugs that will be covered in any therapeutic class. Ongoing cost reduction initiatives and future laws could decrease the coverage and price that we will receive for any approved products. While Medicare beneficiaries are limited to most elderly and certain disabled individual, private payors often follow Medicare coverage policy and payment limitations in setting their own payment rates.

In March 2010, the Affordable Care Act, or ACA became law. The ACA is 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 the healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. Among the provisions of the ACA of importance to our product candidates are the following:

- an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic products;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;
- expansion of healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers, and enhanced penalties for noncompliance;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 70% point-of-sale discounts off negotiated prices;
- extension of manufacturers' Medicaid rebate liability;
- expansion of eligibility criteria for Medicaid programs;
- expansion of the entities eligible for discounts under the Public Health Service Act's pharmaceutical pricing program;
- new requirements to report to CMS financial arrangements with physicians, as defined by such law, and teaching hospitals;
- a new requirement to annually report to FDA drug samples that manufacturers and distributors provide to physicians; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

There remain challenges to certain aspects of the ACA, and we expect there will be additional challenges and amendments to the ACA in the future. Since January 2017, President Trump has signed two Executive Orders and other directives designed to eliminate the implementation of certain provisions of the ACA or otherwise circumvent some of the requirements for health insurance mandated by the ACA. Concurrently, Congress has considered legislation that would repeal or repeal and replace all or part of the ACA. While Congress has not passed comprehensive repeal legislation, several bills affecting the implementation of certain taxes under the ACA have been signed into law. The Tax Cuts and Jobs Act was enacted, which, among other things, included a provision which repealed, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate". In addition, the 2020 federal spending package permanently eliminated, effective January 1, 2020, the

ACA-mandated “Cadillac” tax on certain high-cost employer-sponsored health insurance plans and the medical device excise tax on non-exempt medical devices and, effective January 1, 2021, also eliminates the health insurer tax. On December 14, 2018, a Texas U.S. District Court Judge ruled that the ACA is unconstitutional in its entirety because the “individual mandate” was repealed by Congress as part of the Tax Cuts and Jobs Act. Additionally, on December 18, 2019, the U.S. Court of Appeals for the 5th Circuit upheld the District Court ruling that that the individual mandate was unconstitutional and remanded the case back to the District Court to determine whether the remaining provisions of the ACA are invalid as well. It is unclear how this decision, future decisions, subsequent appeals, and other efforts to repeal and replace the ACA will impact the ACA.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted. These new laws may result in additional reductions in Medicare and other healthcare funding.

There has also been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several Congressional inquiries and proposed bills designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug products. For example, the Trump administration’s budget proposal for fiscal year 2021 includes a $135 billion allowance to support legislative proposals seeking to reduce drug prices, increase competition, lower out-of-pocket drug costs for patients, and increase patient access to lower-cost generic and biosimilar drugs. Further, the Trump administration released a “Blueprint” to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase drug manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products, and reduce the out of pocket costs of drug products paid by consumers. The Department of Health and Human Services, or HHS, has solicited feedback on some of these measures and has implemented others under its existing authority. While some of these and other measures may require additional authorization to become effective. Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. Individual states in the United States have also become increasingly active in passing legislation and implementing regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

We expect that the ACA, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we will receive for any approved product. Any reduction in payments from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our products.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals, if any, 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 conditions and other requirements.

We are subject to anti-corruption laws, as well as export control laws, customs laws, sanctions laws and other laws governing our operations. If we fail to comply with these laws, we could be subject to civil or criminal penalties, other remedial measures and legal expenses, which could adversely affect its business, results of operations and financial condition.

Our operations are subject to anti-corruption laws, including the Foreign Corrupt Practices Act, or FCPA, and other anti-corruption laws that apply in countries where we do business and may do business in the future. The FCPA and these other laws generally prohibit us, our officers and employees and intermediaries from bribing, being bribed or making other prohibited payments to government officials or other persons to obtain or retain business or gain some other business advantage. We may in the future operate in jurisdictions that pose a high risk of potential FCPA violations, and may participate in collaborations and relationships with third parties whose actions could potentially subject us to liability under the FCPA or local anti-corruption laws. In addition, we cannot predict the nature, scope or effect of future regulatory requirements to which its international operations might be subject or the manner in which existing laws might be administered or interpreted.

We are also subject to other laws and regulations governing our international operations, including regulations administered by the U.S. government and authorities in the European Union or the United Kingdom, including applicable export control

regulations, economic sanctions on countries and persons, customs requirements and currency exchange regulations, which we collectively refer to as Trade Control Laws.

There is no assurance that we will be completely effective in ensuring our compliance with all applicable anti-corruption laws, including the FCPA or other legal requirements, including Trade Control Laws. If we are not in compliance with the FCPA and other anti-corruption laws or Trade Control Laws, we may be subject to criminal and civil penalties, disgorgement and other sanctions and remedial measures, and legal expenses, which could have an adverse impact on our business, financial condition, results of operations and liquidity. Likewise, any investigation of any potential violations of the FCPA, other anti-corruption laws or Trade Control Laws by U.S. or other authorities could also have an adverse impact on our reputation, business, results of operations and financial condition.

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

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

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

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

- our inability to recruit and retain adequate numbers of effective sales and marketing personnel;
- the inability of sales personnel to obtain access to physicians or educate adequate numbers of physicians on the benefits of prescribing any future products; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we enter into arrangements with third parties to perform sales, marketing and distribution services, our product revenue or the profitability of these product revenue to us may 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 may have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates.

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

The development and commercialization of new drug products is highly competitive. We face competition with respect to our current product candidates, and will face competition with respect to any product candidates that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of cancer, such as ccRCC. 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.

Our lead product candidate, mavorixafor, is in clinical development for the treatment of WHIM syndrome, SCN and WM, respectively. We are aware of other companies that are developing CXCR4 inhibitors that are in a similar stage of development as mavorixafor, including Eli Lilly, Pfizer, Bristol-Myers Squibb, or BMS, BioLineRx, Noxxon, Upsher-Smith, Polyphor and Glycomimetics. To our knowledge, there do not appear to be any competitors with programs in development for WHIM syndrome or SCN. With respect to WM, the Dana Farber Cancer Institute has completed enrollment for a trial to study the BMS CXCR4 antibody (IV infusion) in the treatment of WM patients with CXCR4 mutations. In WM, there are several treatment approaches currently being developed, including targeted therapies and immunotherapies (as monotherapies and combination therapies), chemotherapy, stem cell transplantation, and cancer vaccines. With the exception of the BMS monoclonal antibody, none to our knowledge have a mechanism of action that interacts with the CXCR4 receptor.

There are a variety of available drug therapies marketed for cancer. In many cases, these drugs are administered in combination to enhance efficacy. 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. Insurers and other third-party payors may also encourage the use of generic products. We expect that if our product candidates are approved, they will be priced at a significant premium over competitive generic products. This may make it difficult for us to achieve our business strategy of using our product candidates in combination with existing therapies or replacing existing therapies with our product candidates.

Our competitors may develop products that are more effective, have a better safety profile, are more convenient or less costly than any that we are developing or that would render our product candidates obsolete or non-competitive. Our competitors may also obtain marketing approval from the FDA or other regulatory authorities for their products sooner than we may obtain approval for our product candidates, which could result in our competitors establishing a strong market position before we are able to enter the market.

Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties may 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.

We intend to market mavorixafor and our other product candidates outside of the United States, and if we do, we will be subject to the risks of doing business outside of the United States.

Because we intend to market mavorixafor and other product candidates, if approved, outside of the United States, our business is subject to risks associated with doing business outside of the United States. Accordingly, our business and financial results in the future could be adversely affected due to a variety of factors, including:

- failure to develop an international sales, marketing and distribution system for our products;
- changes in a specific country's or region's political and cultural climate or economic condition;
- unexpected changes in foreign laws and regulatory requirements;
- difficulty of effective enforcement of contractual provisions in local jurisdictions;
- inadequate intellectual property protection in foreign countries;
- inadequate data protection against unfair commercial use;
- trade-protection measures, import or export licensing requirements such as Export Administration Regulations promulgated by the United States Department of Commerce and fines, penalties or suspension or revocation of export privileges;
- the effects of applicable foreign tax structures and potentially adverse tax consequences; and
- significant adverse changes in foreign currency exchange rates.

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

The laws and regulations that govern marketing approvals, pricing, coverage and reimbursement for new drug products vary widely from country to country. Current and future legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted and, in some markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval.

Our ability to commercialize mavorixafor or any other product candidate successfully also will depend in part on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from government health administration authorities, private health insurers and other organizations. Government authorities and other third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the U.S. and E.U. healthcare industries and elsewhere is cost containment. Government authorities and other 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 mavorixafor or any other product that we commercialize and, if coverage and reimbursement is available, the level of reimbursement. Reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. Obtaining and maintaining adequate reimbursement for mavorixafor may be particularly difficult because of the higher prices typically associated with drugs directed at smaller populations of patients. In addition, third-party payors are likely to impose strict requirements for reimbursement of a higher priced drug, and any launch of a competitive product is likely to create downward pressure on the price initially charged. If reimbursement is not available or is available only to a limited degree, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval.

There may be significant delays in obtaining coverage and reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the drug is approved by the applicable regulatory authority. Moreover, eligibility for coverage and reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, intellectual property, manufacturing, sale and distribution expenses. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs, and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. In the United States, third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. In the European Union, reference pricing systems and other measures may lead to cost containment and reduced prices. Our inability to promptly obtain coverage and adequate reimbursement rates from both government-funded and private payors for any approved products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to develop product candidates and commercialize products and our overall financial condition.

Governments outside the United States tend to impose strict price controls, which may adversely affect our revenues, if any.

In some countries, particularly the countries of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be harmed, possibly materially.

Product liability lawsuits against us could cause us to incur substantial liabilities and could limit the commercialization of any product candidates we may develop.

We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical trials and will face an even greater risk with respect to commercial sales of any products that we may develop. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- reduced resources of our management to pursue our business strategy;
- decreased demand for any products that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend any related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue;
- increased insurance costs; and
- the inability to commercialize any products that we may develop.

Although we maintain clinical trial insurance coverage, it may not be adequate to cover all liabilities that we may incur. We anticipate that we will need to increase our insurance coverage as we continue clinical trials or begin commercialization of any products. Insurance coverage is increasingly expensive. We may not be able to obtain or maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

Risks Related to Our Dependence on Third Parties

We have minimal experience manufacturing our product candidates on a large clinical or commercial scale and have no manufacturing facility. We are currently dependent on a single third party manufacturer for the manufacture of mavorixafor, the active pharmaceutical ingredient, or API, and a single manufacturer of mavorixafor finished drug product capsules. If we experience problems with these third parties, the manufacturing of mavorixafor could be delayed, which could harm our results of operations.

We do not own or operate facilities for the manufacture of mavorixafor or any other product candidate. We currently have no plans to build our own clinical or commercial scale manufacturing capabilities. We currently work exclusively with one manufacturer for the production of mavorixafor, the active pharmaceutical ingredient, or API, and a single manufacturer of mavorixafor finished drug product capsules. To meet our projected needs for clinical supplies to support our activities through regulatory approval and commercial manufacturing, the manufacturer with whom we currently work will need to increase its frequency and/or scale of production or we will need to find additional or alternative manufacturers. We have not yet secured alternate suppliers in the event the current manufacturer we utilize is unable to meet demand, or if otherwise we experience any problems with them. If such problems arise and we are unable to arrange for alternative third-party manufacturing sources, we are unable to find an alternative third party capable of reproducing the existing manufacturing method or we are unable to do so on commercially reasonable terms or in a timely manner, we may not be able to complete development of our product candidates, or market or distribute them.

Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured our product candidates ourselves, including reliance on the third party for regulatory compliance and quality assurance, the possibility of breach of the manufacturing agreement by the third party because of factors beyond our control (including a failure to synthesize and manufacture our product candidates or any products that we may eventually commercialize in accordance with our specifications), and the possibility of termination or nonrenewal of the agreement by the third party, based on its own business priorities, at a time that is costly or damaging to us. In addition, the FDA and other regulatory authorities require that our product candidates and any products that we may eventually commercialize be manufactured according to cGMP and similar foreign standards. Drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and some state agencies, and are subject to periodic unannounced inspections for compliance with

cGMP requirements. Changes to the manufacturing process are strictly regulated and often require prior FDA or other regulatory authority approval before being implemented. FDA requirements 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, the manufacturers must continue to expend time, money and effort in the areas of production and quality control to maintain cGMP compliance. Any failure by our third-party manufacturers to comply with cGMP or failure to scale up manufacturing processes, including any failure to deliver sufficient quantities of product candidates or products if they are approved in a timely manner, could lead to a delay in, or failure to obtain, regulatory approval of any of our product candidates. In addition, such failure could be the basis for the FDA to issue a warning letter, withdraw approvals for product candidates previously granted to us, or take other regulatory or legal action, including recall or seizure, total or partial suspension of production, suspension of ongoing clinical trials, refusal to approve pending applications or supplemental applications, detention of product, refusal to permit the import or export of products, injunction, or imposing civil and criminal penalties.

Our current manufacturer and any future manufacturers may not be able to manufacture our product candidates at a cost or in quantities or in a timely manner necessary to make commercially successful products. If we successfully commercialize any of our product candidates, we may be required to establish large-scale commercial manufacturing capabilities. In addition, as our drug development pipeline increases and matures, we will have a greater need for clinical study and commercial manufacturing capacity. We have no experience manufacturing pharmaceutical products on a commercial scale and some of these manufacturers will need to increase their scale of production to meet our projected needs for commercial manufacturing, the satisfaction of which may not be met on a timely basis.

We rely on third-party CROs to conduct our preclinical studies and clinical trials. If these CROs do not successfully carry out their contractual duties or meet expected deadlines, we may not be able to obtain regulatory approval for or commercialize our product candidates and our business could be substantially harmed.

We have relied upon and plan to continue to rely upon third-party contract research organizations, or CROs, and clinical data management organizations to monitor and manage data for our ongoing preclinical and clinical programs. Although we control only certain aspects of their activities, we are responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol and legal, regulatory and scientific standards, and our reliance on the CROs does not relieve us of our regulatory responsibilities. We also rely on third parties to conduct our preclinical studies in accordance with Good Laboratory Practice, or GLP, requirements and the Laboratory Animal Welfare Act of 1966 requirements. We, our CROs and our clinical trial sites are required to comply with regulations and current Good Clinical Practices, or GCP, and comparable foreign requirements to ensure that the health, safety and rights of patients are protected in clinical trials, and that data integrity is assured. Regulatory authorities ensure compliance with GCP requirements through periodic inspections of trial sponsors and trial sites. If we, any of our CROs or our clinical trial sites fail to comply with applicable GCP requirements, the clinical data generated in our clinical trials or a specific site may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications.

Our CROs are not our employees, and except for remedies available to us under our agreements with such CROs, we cannot control whether or not they devote sufficient time and resources to our ongoing clinical and preclinical programs. If CROs do not successfully carry out their contractual obligations or meet expected timelines or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols, regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. As a result, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenues could be delayed.

Disruptions in our supply chain could delay the commercial launch of our product candidates.

Any significant disruption in our supplier relationships could harm our business. We currently rely on a single source supplier of mavorixafor, as well a single supplier for the finished product capsules for mavorixafor. If either of these single source suppliers suffers a major natural or man-made disaster at its manufacturing facility, we would not be able to manufacture mavorixafor on a commercial scale until a qualified alternative supplier is identified. Although alternative sources of supply exist, the number of third party suppliers with the necessary manufacturing and regulatory expertise and facilities is limited, and it could be expensive and take a significant amount of time to arrange for alternative suppliers. Any significant delay in the supply of a product candidate or its key materials for an ongoing clinical study could considerably delay completion of our clinical studies, product testing and potential regulatory approval of our product candidates. If we or our manufacturers are unable to purchase these key

materials after regulatory approval has been obtained for our product candidates, the commercial launch of our product candidates would be delayed, which would impair our ability to generate revenues from the sale of our product candidates.

Our employees, principal investigators, CROs and consultants may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could have a material adverse effect on our business.

We are exposed to the risk that our employees, principal investigators, CROs and consultants may engage in fraudulent conduct or other illegal activity. Misconduct by these parties could include intentional failures to comply with FDA regulations or similar regulations of comparable foreign regulatory authorities, to provide accurate information to the FDA or comparable foreign regulatory authorities, to comply with manufacturing standards we have established, to 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, to report financial information or data accurately or to disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Employee or third party 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 employee misconduct, and the precautions we take to detect and prevent this activity, such as employee training, 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 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.

We have established, and may seek to selectively establish in the future, collaborations, and, if we are unable to establish them on commercially reasonable terms, 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 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 candidates.

Future development and potential commercialization of mavorixafor in ccRCC will be pursued only as part of a potential strategic collaboration. In addition, we have entered into an agreement with Abbisko Therapeutics to develop and commercialize mavorixafor, in combination with checkpoint inhibitors or other agents in Greater China for oncology indications. No assurance can be given, however, that any current or future strategic collaboration will prove to be successful.

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.

If we decide to expand or modify one or our existing collaboration agreements or to collaborate with a new third party in connection with any of our development programs or product candidates, 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 program or the product candidate for which we are seeking to collaborate, reduce or delay our development program or one or more of our other development programs, delay our 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 such collaborations for the development and commercialization of our product candidates. If those collaborations are not successful, we may not be able to capitalize on the market potential of our product candidates.

We have, and may selectively seek in the future, third-party collaborators for the development and commercialization of our product candidates. Our likely collaborators for any collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. If we 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 revenue 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 pose many risks to us, including that:

- 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 product candidates or products 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;
- collaborators with marketing and distribution rights to one or more product candidates or products may not commit sufficient resources to the marketing and distribution of such drugs;
- 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 product candidates or products or that result in costly litigation or arbitration that diverts management attention and resources;
- we may lose certain valuable rights under circumstances identified in our collaborations if we undergo a change of control;
- 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; and
- collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. In addition, 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 under such collaboration could be delayed, diminished or terminated.

We may engage in future acquisitions or in-licenses of technology that could disrupt our business, cause dilution to our stockholders and harm our financial condition and operating results.

While we currently have no specific plans to acquire any other businesses or in-license any additional products or technology, we may, in the future, make acquisitions or licenses of, or investments in, companies, products or technologies that we believe are a strategic or commercial fit with our current product candidates and business or otherwise offer opportunities for us. In connection with these acquisitions or investments, we may:

- issue stock that would dilute our stockholders' percentage of ownership;
- expend cash;
- incur debt and assume liabilities; and
- incur amortization expenses related to intangible assets or incur large and immediate write-offs.

We also may be unable to find suitable acquisition or license candidates and we may not be able to complete acquisitions or licenses on favorable terms, if at all. If we do complete an acquisition or license, we cannot assure you that it will ultimately strengthen our competitive position or that it will not be viewed negatively by customers, financial markets or investors. Further, the Merger poses, and future acquisitions or licenses could also pose, numerous additional risks to our operations, including:

- problems integrating the purchased or licensed business, products or technologies;
- increases to our expenses
- the failure to have discovered undisclosed liabilities of the acquired or licensed asset or company;
- diversion of management's attention from their day-to-day responsibilities;
- harm to our operating results or financial condition;
- entrance into markets in which we have limited or no prior experience; and
- potential loss of key employees, particularly those of the acquired entity.

We may not be able to complete one or more acquisitions or effectively integrate the operations, products or personnel gained through any such acquisition without a material adverse effect on our business, financial condition and results of operations.

Risks Related to Our Intellectual Property

Recent laws and rulings by U.S. courts make it difficult to predict how patents will be issued or enforced in our industry.

Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may have a significant impact on our ability to protect our technology and enforce our intellectual property rights.

There have been numerous recent changes to the patent laws and to the rules of the United States Patent and Trademark Office, or USPTO, which may have a significant impact on our ability to protect our technology and enforce our intellectual property rights. For example, the Leahy-Smith America Invents Act, or AIA, which was signed into law in 2011, includes a transition from a "first-to-invent" system to a "first-to-file" system, and changes the way issued patents are challenged. Certain changes, such as the institution of inter partes review proceedings, that allow third parties to challenge newly issued patents, came into effect on September 16, 2012. The burden of proof required for challenging a patent in these proceedings is lower than in district court litigation, and patents in the biologics and pharmaceuticals industry have been successfully challenged using these new post-grant challenges. In addition, the U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in specified circumstances or weakening the rights of patent owners in specified situations. Depending on decisions by the U.S. Congress, the federal courts, and the USPTO, these substantive changes to patent law associated with the AIA may further weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future, all of which could harm our business.

Furthermore, the patent positions of companies engaged in the development and commercialization of biologics and pharmaceuticals are particularly uncertain. Two cases involving diagnostic method claims and "gene patents" have recently been decided by the Supreme Court. On March 20, 2012, the Supreme Court issued a decision in Mayo Collaborative Services v. Prometheus Laboratories, Inc., or Prometheus, a case involving patent claims directed to measuring a metabolic product in a

patient to optimize a drug dosage amount for the patient. According to the Supreme Court, the addition of well-understood, routine or conventional activity such as "administering" or "determining" steps was not enough to transform an otherwise patent ineligible natural phenomenon into patent eligible subject matter. On July 3, 2012, the USPTO issued guidance indicating that process claims directed to a law of nature, a natural phenomenon or an abstract idea that do not include additional elements or steps that integrate the natural principle into the claimed invention such that the natural principle is practically applied and the claim amounts to significantly more than the natural principle itself should be rejected as directed to non-statutory subject matter. On June 13, 2013, the Supreme Court issued its decision in Association for Molecular Pathology v. Myriad Genetics, Inc., or Myriad, a case involving patent claims held by Myriad Genetics, Inc. relating to the breast cancer susceptibility genes BRCA1 and BRCA2. Myriad held that isolated segments of naturally occurring DNA, such as the DNA constituting the BRCA1 and BRCA2 genes, is not patent eligible subject matter, but that complementary DNA, which is an artificial construct that may be created from RNA transcripts of genes, may be patent eligible.

We cannot assure you that our efforts to seek patent protection for our technology and products will not be negatively impacted by the changes described above, future rulings in district court cases or changes in guidance or procedures issued by the USPTO. We cannot fully predict what impact the Supreme Court's decisions may have on the ability of life science companies to obtain or enforce patents relating to their products and technologies in the future.

Moreover, although the Supreme Court has held in Myriad that isolated segments of naturally occurring DNA are not patent-eligible subject matter, certain third parties could allege that activities that we may undertake infringe other gene-related patent claims, and we may deem it necessary to defend ourselves against these claims by asserting non-infringement and/or invalidity positions, or pay to obtain a license to these claims. In any of the foregoing or in other situations involving third-party intellectual property rights, if we are unsuccessful in defending against claims of patent infringement, we could be forced to pay damages or be subjected to an injunction that would prevent us from utilizing the patented subject matter. Such outcomes could harm our business.

If we are unable to protect our intellectual property rights, our competitive position could be harmed.

We depend on our ability to protect our proprietary technology. We rely on trade secret, patent, copyright and trademark laws, and confidentiality, licensing and other agreements with employees and third parties, all of which offer only limited protection. Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary technology and products. Where we have the right to do so under our license agreements, we seek to protect our proprietary position by filing patent applications in the United States and abroad related to our novel technologies and products that are important to our business.

The patent positions of biotechnology and pharmaceutical companies generally are highly uncertain, involve complex legal and factual questions and have in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patents, including those patent rights licensed to us by third parties, are highly uncertain.

The steps we have taken to police and protect our proprietary rights may not be adequate to preclude misappropriation of our proprietary information or infringement of our intellectual property rights, both inside and outside the United States. The rights already granted under any of our currently issued patents and those that may be granted under future issued patents may not provide us with the proprietary protection or competitive advantages that we are seeking. If we are unable to obtain and maintain patent protection for our technology and products, or if the scope of the patent protection obtained is not sufficient, our competitors could develop and commercialize technology and products similar or superior to ours, and our ability to successfully commercialize our technology and products may be adversely affected.

With respect to patent rights, we do not know whether any of the pending patent applications for any of our product candidates will result in the issuance of patents that protect our technology or products, or which will effectively prevent others from commercializing competitive technologies and products. Our pending applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Further, the examination process may require us or our licensors to narrow the claims, which may limit the scope of patent protection that may be obtained. Although our license agreement with Genzyme includes a number of issued patents that are exclusively licensed to us, the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, issued patents that we own or have licensed from third parties may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in the loss of patent protection, the narrowing of claims in such patents, or the invalidity or unenforceability of such

patents, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection for our technology and products. Protecting against the unauthorized use of our patented technology, trademarks and other intellectual property rights is expensive, difficult and may, in some cases, not be possible. In some cases, it may be difficult or impossible to detect third party infringement or misappropriation of our intellectual property rights, even in relation to issued patent claims, and proving any such infringement may be even more difficult.

We could be required to incur significant expenses to obtain our intellectual property rights, and we cannot ensure that we will obtain meaningful patent protection for our product candidates.

The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. In addition, it is also possible that we will fail to identify patentable aspects of further inventions made in the course of our development and commercialization activities before they are publicly disclosed, making it too late to obtain patent protection on them. Further, 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. We expect to seek extensions of patent terms where these are available in any countries where we are prosecuting patents. This includes in the United States under the Drug Price Competition and Patent Term Restoration Act of 1984, which permits a patent term extension of up to five years beyond the expiration of a patent that covers an approved product where the permission for the commercial marketing or use of the product is the first permitted commercial marketing or use, and as long as the remaining term of the patent does not exceed 14 years. However, the applicable authorities, including the FDA in the United States, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or may grant more limited extensions than we request. If this occurs, our competitors may be able to take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case. 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, and these foreign laws may also be subject to change. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or in some cases not at all. Therefore, we cannot be certain that we or our licensors were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions.

In March 2013, the United States transitioned to a ‘first to file’ system in which the first inventor to file a patent application will be entitled to the patent. Third parties are allowed to submit prior art prior to the issuance of a patent by the USPTO and may become involved in post-grant review or derivation proceedings for applications filed on or after March 16, 2013, interference proceedings for applications filed before March 16, 2013, ex parte reexamination, or inter partes review 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, which could adversely affect our competitive position with respect to third parties.

Obtaining and maintaining our patent protection depends on compliance with various procedural, documentary, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees on any issued patent are due to be paid to the USPTO, and foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other requirements during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we or our licensors fail to maintain the patents and patent applications covering our product candidates, our competitors might be able to enter the market, which would have a material adverse effect on our business.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products, including generics or biosimilars. 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 intellectual property, which could be expensive, time consuming and unsuccessful.

In addition to the possibility of litigation relating to infringement claims asserted against it, we may become a party to other patent litigation and other proceedings, including inter partes review proceedings, post-grant review proceedings, derivation proceedings declared by the USPTO and similar proceedings in foreign countries, regarding intellectual property rights with respect to our current or future technologies or product candidates or products. The cost to us of any patent litigation or other proceeding, even if resolved in our favor, could be substantial. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their substantially greater financial resources. Patent litigation and other proceedings may also absorb significant management time. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could impair our ability to compete in the marketplace.

Competitors may infringe or otherwise violate our intellectual property, including patents that may issue to or be licensed by us. As a result, we may be required to file claims in an effort to stop third-party infringement or unauthorized use. Any such claims could provoke these parties to assert counterclaims against us, including claims alleging that we infringe their patents or other intellectual property rights. This can be prohibitively expensive, particularly for a company of our size, and time-consuming, and even if we are successful, any award of monetary damages or other remedy we may receive may not be commercially valuable. In addition, in an infringement proceeding, a court may decide that our asserted intellectual property is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our intellectual property does not cover its technology. An adverse determination in any litigation or defense proceedings could put our intellectual property at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing.

If the breadth or strength of our patent or other intellectual property rights is compromised or threatened, it could allow third parties to commercialize our technology or products or result in our inability to commercialize our technology and products without infringing third-party intellectual property rights. Further, third parties may be dissuaded from collaborating with us.

Interference or derivation proceedings brought by the USPTO or its foreign counterparts may be necessary to determine the priority of inventions with respect to our patent applications, and we may also become involved in other proceedings, such as re-examination proceedings, before the USPTO or its foreign counterparts. Due to the substantial competition in the pharmaceutical space, the number of such proceedings may increase. This could delay the prosecution of our pending patent applications or impact the validity and enforceability of any future patents that we may obtain. In addition, any such litigation, submission or proceeding may be resolved adversely to us and, even if successful, may result in substantial costs and distraction to our management.

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. Moreover, intellectual property law relating to the fields in which we operate is still evolving and, consequently, patent and other intellectual property positions in our industry are subject to change and are often uncertain. We may not prevail in any of these suits or other efforts to protect our technology, and the damages or other remedies awarded, if any, may not be commercially valuable. During the course of this type of litigation, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, the market price for our common stock could be significantly harmed.

If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose intellectual property rights that are important to our business.

We are a party to several license agreements and may need to obtain additional licenses from others to advance our research and development activities or allow the commercialization of our current product candidates and any that we may identify and pursue in the future. Our currently license agreements impose, and we expect that future license agreements will impose, various development, diligence, commercialization, and other obligations on us. In spite of our efforts, our licensors might conclude that we have materially breached our obligations under such license agreements and might therefore terminate the license agreements, thereby removing or limiting our ability to develop and commercialize products and technology covered by these license agreements. If these in-licenses are terminated, or if the underlying patents fail to provide the intended exclusivity, competitors or other third parties may gain the freedom to seek regulatory approval of, and to market, products identical to ours and we may be required to cease our development and commercialization of our product candidates. Any of the foregoing could have a material adverse effect on our competitive position, business, financial conditions, results of operations, and prospects.

Moreover, disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our product candidates, technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.

In addition, the agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations, and prospects. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates, which could have a material adverse effect on our business, financial conditions, results of operations, and prospects.

From time to time, we may need to rely on licenses to proprietary technologies, which may be difficult or expensive to obtain or we may lose certain licenses which may be difficult to replace.

We may need to obtain licenses to patents and other proprietary rights held by third parties to develop, manufacture and market our product candidates. If we are unable to timely obtain these licenses on commercially reasonable terms and maintain these licenses, our ability to commercially market our product candidates may be inhibited or prevented, which could have a material adverse effect on our business, results of operations, financial condition and cash flows.

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 to develop, manufacture, market and sell our product candidates, and to use our proprietary technologies without infringing the proprietary rights of third parties. We may become party to, or threatened with, future adversarial proceedings or litigation regarding intellectual property rights with respect to our products and technology, including interference and various post grant proceedings before the USPTO, non-U.S. opposition proceedings, and German nullity proceedings. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future.

As a result of any such infringement claims, or to avoid potential claims, we may choose or be compelled to seek intellectual property licenses from third parties. These licenses may not be available on acceptable terms, or at all. Even if we are able to obtain a license, the license would likely obligate us to pay license fees or royalties or both, and the rights granted to us likely would be nonexclusive, which would mean that our competitors also could obtain licenses to the same intellectual property. Ultimately, we could be prevented from commercializing a product candidate or technology or be forced to cease some aspect of our business operations if, as a result of actual or threatened infringement claims, we are unable to enter into licenses of the relevant intellectual property on acceptable terms. Further, if we attempt to modify a product candidate or technology or to develop alternative methods or products in response to infringement claims or to avoid potential claims, we could incur substantial costs, encounter delays in product introductions or interruptions in sales. Ultimately, such efforts could be unsuccessful.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize our product candidates that we may identify. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure.

Parties making claims against us may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation or administrative proceedings, there is a risk that some of our confidential information could be compromised by disclosure. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have material adverse effect on our ability to raise additional funds or otherwise have a material adverse effect on our business, results of operations, financial condition and prospects.

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

Litigation or other legal proceedings relating to intellectual property claims, with or without merit, is unpredictable and generally expensive and time consuming and is likely to divert significant resources from our core business, including distracting our technical and management personnel from their normal responsibilities. 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, 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 and negatively impact our ability to raise additional funds. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities.

We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. Accordingly, despite our efforts, we may not be able to prevent third parties from infringing upon or misappropriating or from successfully challenging our intellectual property rights. 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.

Our trade secrets are difficult to protect and 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 technologies and product candidates, 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, non-competition, non-solicitation, and invention assignment agreements with our employees and consultants that obligate them to assign to us any inventions developed in the course of their work for us. However, we cannot guarantee that we have executed these agreements with each party that may have or have had access to our trade secrets or that the agreements we have executed will provide adequate protection. 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. As a result, we may be forced to bring claims against third parties, or defend claims that they bring against us, to determine ownership of what we regard as our intellectual property. Monitoring unauthorized disclosure is difficult and we do not know whether the procedures that we have followed to prevent such disclosure are or will be adequate. 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 may be less willing or unwilling to protect trade secrets. If any of the technology or information that we protect as 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.

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

Our employees, including members of our senior management, were previously employed at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. All such individuals, including each member of our senior management, executed proprietary rights, non-disclosure and non-competition agreements in connection with such previous employment. 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. We are not aware of any threatened or pending claims related to these matters or concerning the agreements with our senior management, but in the future litigation may be necessary to defend against such 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.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting and defending patents on all of our product candidates throughout the world would be prohibitively expensive. In general, we have sought patent protection of our intellectual property in the following jurisdictions: US, Canada, China, Japan and in countries within Europe via the European Patent Office. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and further, may export otherwise infringing products to territories where we have patent protection, but where enforcement is not as strong as that in the United States. These products may compete with our products in jurisdictions where we do not have any issued patents and our patent claims or other intellectual property rights may not be effective or sufficient to prevent them from so competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents and other intellectual property protection, particularly those relating to biopharmaceuticals, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial cost and divert our efforts and attention from other aspects of our business.

Risks Related to the Regulatory Approval, Marketing and Commercialization of Our Product Candidates

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

Our product candidates and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by comparable authorities in other countries. Failure to obtain marketing approval for a product candidate will prevent us from commercializing the product candidate. We have not received approval to market any of our product candidates from regulatory authorities in any jurisdiction. Securing regulatory approval requires the submission of extensive preclinical and clinical data and supporting information to the various regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. Securing regulatory approval also requires the submission of information about the product manufacturing process to, and inspection of

manufacturing facilities by, the relevant regulatory authority. Our product candidates may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude us from obtaining marketing approval or prevent or limit commercial use.

The process of obtaining marketing approvals, both in the United States and elsewhere, is expensive, may take many years and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. We cannot assure you that we will ever obtain any marketing approvals in any jurisdiction. Changes in marketing approval requirements 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 and comparable authorities in other countries have substantial discretion in the review and approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require additional nonclinical or other studies, and clinical trials. In addition, varying interpretations of the data obtained from preclinical testing and clinical trials could delay, limit or prevent marketing approval of a product candidate. Additionally, any marketing approval that we ultimately may obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable.

Failure to obtain regulatory approval in international jurisdictions would prevent our product candidates from being marketed abroad.

In order to market and sell our products in the European Union and many other jurisdictions, we must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The regulatory review and approval process outside the United States generally includes all of the risks associated with obtaining FDA approval, but can involve additional testing and clinical trial requirements and in-country regulatory and/or legal representation. We may need to partner with third parties in order to obtain approvals outside the United States. In addition, in many countries worldwide, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any market. If we are unable to obtain approval of mavorixafor or any other product candidate by regulatory authorities in the European Union or other countries, the commercial potential of those product candidates may be significantly diminished and our business prospects could decline.

A Breakthrough Therapy Designation By The FDA For Our Product Candidates May Not Lead To A Faster Development Or Regulatory Review Or Approval Process, And It Does Not Increase The Likelihood That Our Product Candidates Will Receive Marketing Approval.

We have obtained breakthrough therapy designation for mavorixafor for the treatment of adult patients with WHIM and we may pursue that designation for other product candidates as well. A breakthrough therapy is defined as a product that is intended, alone or in combination with one or more other drugs, to treat a serious or life- threatening condition, and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. For product candidates that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Such designation also offers an intensive and efficient review involving FDA senior managers and experienced review and regulatory health project management staff across disciplines. A breakthrough therapy designation affords the possibility of rolling review, enabling the FDA to review portions of our marketing application before submission of a complete application, and possibly, priority review.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe that our product candidates meet the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a breakthrough therapy designation for a product candidate may not result in a faster development process, review or approval compared to products considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that the products no longer meet the conditions for qualification.

It is possible that we may not be able to obtain or maintain orphan drug designation or exclusivity for our drug candidates.

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for the treatment or prevention of rare diseases or conditions with relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, or the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition, which is defined as a patient population of fewer than 200,000 individuals in the United States. We received orphan drug designation from the FDA for mavorixafor for the treatment of WHIM syndrome in October 2018, and from the EMA in July 2019. If a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a seven-year period of marketing exclusivity, which precludes the FDA from approving another marketing application for the same drug for the same indication during that time period with some exceptions. A similar provision in the European Union allows 10 years of exclusivity in Europe. The European exclusivity period can be reduced to six years if a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable so that marketing exclusivity is no longer justified. Orphan drug exclusivity may be lost in both the U.S. and Europe under certain situations, such as the inability of the holder of the orphan drug designation to produce sufficient quantities of the drug to meet the needs of patients with the rare disease or condition or for certain other reasons.

Even if our product candidates receive regulatory approval, they may still face future development and regulatory difficulties and any approved products will be subject to extensive post-approval regulatory requirements.

If we obtain regulatory approval for a product candidate, it would be subject to extensive ongoing requirements by the FDA and comparable foreign regulatory authorities governing the manufacture, quality control, further development, labeling, packaging, storage, distribution, safety surveillance, import, export, advertising, promotion, recordkeeping and reporting of safety and other post-market information. The safety profile and efficacy of any product will continue to be closely monitored by the FDA and comparable foreign regulatory authorities after approval. If the FDA or comparable foreign regulatory authorities become aware of new safety information after approval of any of our product candidates, these regulatory authorities may require labeling changes or the FDA may require establishment of a Risk Evaluation Mitigation Strategy, or REMS, or similar strategy, impose significant restrictions on a product's indicated uses or marketing, impose ongoing requirements for potentially costly post-approval studies or post-market surveillance. Progress reports are required at quarterly intervals, every six months and at annual intervals depending upon the country, and more frequently if serious adverse events occur.

In addition, manufacturers of drugs and their facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP regulations. If a regulatory agency discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory agency may impose restrictions on that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing. If we, our product candidates or the manufacturing facilities for our product candidates fail to comply with cGMPs and other applicable regulatory requirements, the FDA may, among other things:

- issue warning letters;
- request modifications to promotional materials or require us to provide corrective information to healthcare practitioners;
- require us to enter into a consent decree, which can include imposition of various fines, reimbursements for inspection costs, required due dates for specific actions and penalties for noncompliance;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve pending applications or supplements to applications filed by us;
- suspend or impose restrictions on operations, including costly new manufacturing requirements; or
- seize or detain products, refuse to permit the import or export of products, or require us to initiate a product recall.

The occurrence of any event or penalty described above may inhibit our ability to commercialize our products and generate revenue.

Any product candidate for which we obtain marketing approval could be subject to marketing 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.

Any product candidate for which we obtain marketing approval 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, quality assurance and corresponding maintenance of records and documents and 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 medicine. The FDA closely regulates the post-approval marketing and promotion of drugs to ensure that they 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 and/or promotion.

In addition, later discovery of previously unknown problems with our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in, among other things:

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions on the labeling, marketing, distribution or use of a product;
- requirements to conduct post-approval 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; and
- injunctions or the imposition of civil or criminal penalties.

Risks Related to Our Business Operations, Employee Matters and Managing Growth

We currently have a limited number of employees and our future success depends on our ability to retain our executive officers and to attract, retain and motivate qualified personnel.

Because of the specialized scientific and managerial nature of our business, we rely heavily on our ability to attract and retain qualified scientific, technical and managerial personnel. We are highly dependent upon members of our current management team, including Paula Ragan, Ph.D., our Chief Executive Officer, the loss of whose services may adversely impact the achievement of our objectives. Although we have an employment agreement with Dr. Ragan, this agreement is at-will and does not prevent her from terminating her employment with us at any time by providing the requisite advance notice.

Our success will depend on our ability to retain our management team and other key employees, and to attract and retain qualified personnel in the future. The loss of the services of certain members of our senior management or key employees could prevent or delay the implementation and completion of our strategic objectives, or divert management's attention to seeking qualified replacements The competition for qualified personnel in the pharmaceutical field is intense and we cannot guarantee that we will be able to retain our current personnel or attract and retain new qualified personnel necessary for the development of our business or to recruit suitable replacement personnel.

We will need to grow the size of our organization, and we may experience difficulties in managing this growth.

As of December 31, 2019, we had 58 full-time employees. As our development and commercialization plans and strategies develop, or as a result of any future acquisitions, we will need additional managerial, operational, development, sales, marketing, financial and other resources. Our management, personnel and systems currently in place will not be adequate to support this future growth. Future growth would impose significant added responsibilities on our employees, including:

- managing our clinical trials effectively;
- identifying, recruiting, maintaining, motivating and integrating additional employees;
- managing our internal development efforts effectively while complying with our contractual obligations to licensors, contractors and other third parties;
- improving our managerial, development, operational and finance systems; and
- expanding our facilities.

As our operations expand, we will need to manage additional relationships with various strategic collaborators, suppliers and other third parties. Our future financial performance and our ability to commercialize our product candidates and to compete effectively will depend, in part, on our ability to manage any future growth effectively. To that end, we must be able to manage our development efforts and clinical trials effectively and hire, train and integrate additional management, administrative, research and development, and sales and marketing personnel. We may not be able to accomplish these tasks, and our failure to accomplish any of them could prevent us from successfully growing the company.

The pharmaceutical industry is highly competitive and is subject to rapid and significant technological change, which could render our technologies and products obsolete or uncompetitive.

The pharmaceutical industry is highly competitive and is subject to rapid and significant technological change, which could render certain of our products obsolete or uncompetitive. This is particularly true in the development of therapeutics for oncology indications where new products and combinations of products are rapidly being developed that change the treatment paradigm for patients. There is no assurance that our product candidates will be the best, have the best safety profile, be the first to market, or be the most economical to make or use. The introduction of competitive therapies as alternatives to our product candidates could dramatically reduce the value of those development projects or chances of successfully commercializing those product candidates, which could have a material adverse effect on our long-term financial success.

We will compete with companies in the United States and internationally, including major pharmaceutical and chemical companies, specialized CROs, research and development firms, universities and other research institutions. Many of our competitors have greater financial resources and selling and marketing capabilities, greater experience in clinical testing and human clinical trials of pharmaceutical products and greater experience in obtaining FDA and other regulatory approvals than we do. In addition, some of our competitors may have lower development and manufacturing costs.

We rely significantly on information technology and any failure, inadequacy, interruption or security lapse of that technology or loss of data, including any cyber security incidents, could compromise sensitive information related to our business, prevent us from accessing critical information or expose us to liability which could harm our ability to operate our business effectively and adversely affect our business and reputation.

In the ordinary course of our business, we, our contract research organizations and other third parties on which we rely collect and store sensitive data, including legally protected patient health information, personally identifiable information about our employees, intellectual property, and proprietary business information. We manage and maintain our applications and data utilizing on-site systems. These applications and data encompass a wide variety of business-critical information including research and development information and business and financial information.

The secure processing, storage, maintenance and transmission of this critical information is vital to our operations and business strategy. Despite the implementation of security measures, our internal computer systems and those of third parties with which we contract are vulnerable to damage from cyber-attacks, computer viruses, breaches, unauthorized access, interruptions due to employee error or malfeasance or other disruptions, or damage from natural disasters, terrorism, war and telecommunication and electrical failures. Any such event could compromise our networks and the information stored there could be accessed by unauthorized parties, publicly disclosed, lost or stolen. We have measures in place that are designed to detect and respond to such security incidents and breaches of privacy and security mandates. Any such access, disclosure or other loss of information could

result in legal claims or proceedings, liability under laws that protect the privacy of personal information, government enforcement actions and regulatory penalties. Unauthorized access, loss or dissemination could also disrupt our operations, including our ability to conduct research, development and commercialization activities, process and prepare company financial information, manage various general and administrative aspects of our business and damage our reputation, in addition to possibly requiring substantial expenditures of resources to remedy, any of which could adversely affect our business. The loss of clinical trial data could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. In addition, there can be no assurance that we will promptly detect any such disruption or security breach, if at all. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and our research, development and commercialization efforts could be delayed.

Business disruptions could seriously harm our future revenues and financial condition and increase our costs and expenses.

Our operations could be subject to earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics (including but not limited to the novel coronavirus) and other natural or man-made disasters or business interruptions, for which we are predominantly self-insured. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses. We rely on a single third-party manufacturer to provide the active pharmaceutical ingredient for mavorixafor and a single third-party manufacturer to provide fill and finish services for the final drug product formulation of mavorixafor for use in clinical trials. Our ability to obtain clinical supplies of product candidates could be disrupted if the operations of these suppliers are affected by a man-made or natural disaster or other business interruption.

The novel coronavirus outbreak could impact our business.

In December 2019, a novel strain of coronavirus was first reported in China. This virus has now spread to numerous other countries, including the United States and Austria. While we do not currently have significant operations in geographical locations where the coronavirus is currently reported to be most prevalent, we have clinical trial sites either currently approved or pending approval in Italy and South Korea, two of the countries where the coronavirus has demonstrated increased prevalence. We cannot reasonably estimate at this time the impact, if any, that the coronavirus may have on our business or operations in these countries or otherwise. The extent to which the coronavirus impacts our business will depend on future developments, which are highly uncertain and cannot be predicted, including new information which may emerge concerning the severity of the coronavirus and the actions to contain the coronavirus or treat its impact, including on financial markets or otherwise.

Our ability to use our net operating losses to offset future taxable income may be subject to certain limitations.

In general, under Section 382 of the Internal Revenue Code of 1986, as amended, or the Code, a corporation that undergoes an “ownership change” is subject to limitations on its ability to utilize its carryforwards to offset future taxable income. Our existing net operating loss carryforwards, or NOLs, may be subject to limitations arising from previous ownership changes, including in connection with the Merger, and if we undergo a subsequent ownership change, our ability to utilize NOLs could be further limited by Section 382 of the Code. There is also a risk that due to regulatory changes, such as suspensions on the use of NOLs, or other unforeseen reasons, our existing and any future NOLs could expire or otherwise be unavailable to offset future income tax liabilities.

We have not conducted a study to assess whether an ownership change has occurred or whether there have been multiple ownership changes since inception due to the significant complexity and cost associated with such a study.

Our term loan contains restrictions that limit our flexibility in operating our business.

In October 2018, we entered into a loan and security agreement with Hercules Capital, Inc., secured by a lien on substantially all of our assets, including intellectual property. This loan contains various covenants that limit our ability to engage in specified types of transactions. These covenants limit our ability to, among other things:

- sell, transfer, lease or dispose of certain assets;
- incur indebtedness;

- encumber or permit liens on certain assets;
- make certain investments;
- make certain restricted payments, including paying dividends on, or repurchasing or making distributions with respect to, our common stock; and
- enter into certain transactions with affiliates.

The covenants also include maintaining a minimum liquidity amount of the lesser of (i) 125% of outstanding borrowings under the Hercules Loan Agreement and (ii) 100% of our cash and cash equivalents in an account in which Hercules has a first priority security interest. A breach of any of the covenants under the loan and security agreement could result in a default under the loan. Upon the occurrence of an event of default under the loan, the lenders could elect to declare all amounts outstanding, if any, to be immediately due and payable and terminate all commitments to extend further credit. If there are any amounts outstanding that we are unable to repay, the lenders could proceed against the collateral granted to them to secure such indebtedness.

We will incur costs and demands upon management as a result of complying with the laws and regulations affecting public companies.

We will incur significant legal, accounting and other expenses that we did not incur as a private company, including costs associated with public company reporting requirements. We will also incur costs associated with corporate governance requirements, including requirements under the Sarbanes-Oxley Act, as well as rules implemented by the Securities and Exchange Commission, or SEC and Nasdaq. These rules and regulations are expected to increase our legal and financial compliance costs and to make some activities more time consuming and costly. Our executive officers and other personnel will need to devote substantial time regarding operations as a public company and compliance with applicable laws and regulations. These rules and regulations may also make it difficult and expensive for us to obtain and maintain directors' and officers' liability insurance. We have not purchased any key man insurance policies with respect to our executive officers. As a result, it may be more difficult for us to attract and retain qualified individuals to serve on our board of directors or as our executive officers, which may adversely affect investor confidence in us and could cause our business or stock price to suffer.

The impact of the Tax Cuts and Jobs Act on our financial results is not entirely clear and could differ materially from the financial statements included in this Annual Report.

The "Tax Cuts and Jobs Act," or the TCJA, which was enacted in 2017, significantly reforms the U.S. Tax Code. The TCJA, among other things, contained significant changes to corporate taxation, including reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, limitations on the deduction of NOL carryforwards and the elimination of NOL carrybacks, in each case, for losses generated after December 31, 2017 (though any such NOLs may be carried forward indefinitely), limitations on deductions for interest expense and modifications or repeal of many business deductions and credits. Our consolidated financial statements included elsewhere in this Annual Report reflect the effects of the TCJA based on current guidance. However, there remain uncertainties and ambiguities in the application of certain provisions of the TCJA and, as a result, we made certain judgments and assumptions in the interpretation thereof. The U.S. Treasury Department and the Internal Revenue Service, or the IRS, may issue further guidance on how the provisions of the TCJA will be applied or otherwise administered that differs from our current interpretation. In addition, the TCJA could be subject to potential amendments and technical corrections, any of which could materially lessen or increase certain adverse impacts of the legislation on us.

Inadequate funding for the FDA, the SEC and other government agencies could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, the ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory and policy changes. Average review times at the agency have fluctuated in recent years as a result. In addition, government funding of the SEC and other government agencies on which our operations may rely, including those that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years the U.S.

government has shut down several times and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical FDA, SEC and other government employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, in our operations as a public company, future government shutdowns could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

Risks Related to Our Common Stock

Our stock price is expected to continue to be volatile.

The market price of our common stock could continue to be subject to significant fluctuations. Market prices for securities of early-stage pharmaceutical, biotechnology and other life sciences companies have historically been particularly volatile. Some of the factors that may cause the market price of our common stock to fluctuate include:

- our ability or the ability of our collaborators to develop product candidates and conduct clinical trials that demonstrate such product candidates are safe and effective;
- our ability or the ability of our collaborators to obtain regulatory approvals for product candidates, and delays or failures to obtain such approvals;
- failure of any our product candidates to demonstrate safety and efficacy, receive regulatory approval and achieve commercial success;
- failure to maintain our existing third-party license, manufacturing and supply agreements;
- failure by us or our licensors to prosecute, maintain or enforce our intellectual property rights;
- changes in laws or regulations applicable to our current or future product candidates;
- any inability to obtain adequate supply of product candidates or the inability to do so at acceptable prices;
- adverse decisions by regulatory authorities;
- introduction of new or competing products by our competitors;
- failure to meet or exceed financial and development projections that we may provide to the public;
- the perception of the pharmaceutical industry by the public, legislatures, regulators and the investment community;
- announcements of significant acquisitions, strategic collaborations, joint ventures or capital commitments by us or our competitors;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain intellectual property protection for our technologies;
- additions or departures of key personnel;
- significant lawsuits, including intellectual property or stockholder litigation;
- announcements by us of material developments in our business, financial condition and/or operations;
- if securities or industry analysts do not publish research or reports about us, or if they issue an adverse or misleading opinions regarding our business and stock;
- changes in the market valuations of similar companies;
- general market or macroeconomic conditions;
- sales of our common stock or our stockholders in the future;
- trading volume of our common stock;
- adverse publicity relating to our markets generally, including with respect to other products and potential products in such markets;
- changes in the structure of health care payment systems; and

- period-to-period fluctuations in our financial results.

Moreover, the stock markets in general have experienced substantial volatility that has often been unrelated to the operating performance of individual companies. These broad market fluctuations may also adversely affect the trading price of our common stock.

In the past, following periods of volatility in the market price of a company's securities, stockholders have often instituted class action securities litigation against those companies. Such litigation, if instituted, could result in substantial costs and diversion of management attention and resources, which could significantly harm our business, financial condition, results of operations and reputation.

We will be required to raise additional funds to finance our operations; we may not be able to do so when necessary, and/or on acceptable terms.

Our ongoing capital requirements will depend on numerous factors related to the development of our product candidates and the sale of products obtaining regulatory approval, including: the progress and cost of research and development programs and clinical trials; the progress and cost of research and development programs of collaborators; the time and costs expended and required to obtain any necessary or desired regulatory approvals; the costs of ongoing compliance with the FDA and other domestic and foreign regulatory agency requirements; the resources devoted to manufacturing expenditures; the ability to enter into licensing arrangements; the cost of commercialization activities and arrangements, if any, undertaken by us; and, if and when approved, the demand for our products.

We anticipate that we will need to raise additional funds through public or private financings, strategic collaborations or other arrangements. Additional equity financing would be dilutive to our existing stockholders, and debt financing, if available, may involve restrictive covenants. If we raise funds through collaborative or licensing arrangements, we may be required to relinquish, on terms that are not favorable to us, rights to some of our technologies or product candidates that we would otherwise seek to develop or commercialize. Our failure to raise capital when needed could materially harm our business, financial condition and results of operations.

We expect to be heavily reliant on our ability to access funding through capital market transactions. Due to our small public float, market capitalization, limited operating history and lack of revenue, it may be difficult and expensive for us to raise additional funds.

We anticipate that we will be heavily reliant on our ability to raise funds through the issuance of shares of our common stock or securities linked to our common stock. Our ability to raise these funds may be dependent on a number of factors, including the low trading volume and volatile trading price of our shares of common stock and other risk factors described herein. The stocks of small cap companies in the biotechnology sector like us tend to be highly volatile. We expect that the price of our common stock will be highly volatile for the next several years. Even if we expand our portfolio of product candidates, we may never successfully commercialize or monetize our current product candidate or any future product candidate that we may seek to develop.

As a result, we may be unable to access funding through sales of our common stock or other equity-linked securities. Even if we were able to access funding, the cost of capital may be substantial due to our low market cap and our small public float. The terms of any funding we are able to obtain may not be favorable to us and may be highly dilutive to our stockholders. We may be unable to access capital due to unfavorable market conditions or other market factors outside of our control. There can be no assurance that we will be able to raise additional capital when needed. The failure to obtain additional capital when needed would have a material adverse effect on our business.

Raising additional funds by issuing securities may cause dilution to existing stockholders and raising funds through lending and licensing arrangements may restrict our operations or require us to relinquish proprietary rights.

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, grants and license and development agreements in connection with any collaborations. We do not have any committed external source of funds. To the extent that we raise additional capital by issuing equity securities, our existing stockholders' ownership will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing and preferred equity 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 marketing, distribution 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. Any debt financing that we enter into may involve covenants that restrict our operations. These restrictive covenants may include limitations on additional borrowing and specific restrictions on the use of our assets as well as prohibitions on our ability to create liens, pay dividends, redeem our stock or make investments. 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.

We are an "emerging growth company," and a "smaller reporting company" and as a result of the reduced disclosure requirements applicable to emerging growth companies and smaller reporting companies, our common stock may be less attractive to investors.

We are an "emerging growth company," or EGC, as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and may remain an emerging growth company until December 31, 2022. For so long as we remain an EGC, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include:

- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding a supplement to the auditor's report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation; and
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved.

Investors may find our common stock less attractive as a result of our reliance on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

Even following the termination of our status as an emerging growth company, we will be able to take advantage of the reduced disclosure requirements applicable to smaller reporting companies (as that term is defined in Rule 12b-2 of the Exchange Act) and, in particular, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. To the extent that we are no longer eligible to use exemptions from various reporting requirements, we may be unable to realize our anticipated cost savings from these exemptions, which could have a material adverse impact on our operating results.

If securities analysts do not publish research or reports about our business or if they publish negative evaluations of our stock, the price of our stock could decline.

The trading market for our common stock will be influenced, in part, on the research and reports that industry or financial analysts publish about us or our business. Equity research analysts may elect not to provide research coverage of our common stock, and such lack of research coverage may adversely affect the market price of our common stock. In the event we do have equity research analyst coverage, we will not have any control over the analysts or the content and opinions included in their reports. The price of our common stock could decline if one or more equity research analysts downgrade our stock or issue other unfavorable commentary or research. If one or more equity research analysts ceases coverage of us or fails to publish reports on us regularly, demand for our common stock could decrease, which in turn could cause our stock price or trading volume to decline.

We do not anticipate that we will pay any cash dividends in the foreseeable future.

The current expectation is that we will retain our future earnings to fund the development and growth of our business. In addition, the terms of our debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common

stock will be your sole source of gain, if any, for the foreseeable future. We are prohibited from declaring or paying any cash dividends under our existing loan and security agreement with Hercules Capital, Inc.

Sales of a substantial number of shares of our common stock in the public market could cause our stock price to decline.

Sales of a substantial number of shares of our common stock in the public market, or the perception that these sales might occur, could depress the market price of our common stock and could impair our ability to raise capital through the sale of additional equity securities. We are unable to predict the effect that sales, particularly sales by our directors, executive officers, and significant stockholders, may have on the prevailing market price of our common stock.

In addition, we have filed registration statements on Form S-8 registering the issuance of shares of common stock subject to options or other equity awards issued or reserved for future issuance under our equity incentive plans. Shares registered under these registration statements are available for sale in the public market subject to vesting arrangements and exercise of options, as well as Rule 144 in the case of our affiliates.

Additionally, a holder of our common stock has rights, subject to some conditions, to require us to file one or more registration statements covering their shares. These registration rights will terminate upon the earlier to occur of November 17, 2020 or the date on which the holder has sold all shares subject to such registration rights. If we were to register the resale of these shares, they could be freely sold in the public market. If these additional shares are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results or prevent fraud. As a result, stockholders could lose confidence in our financial and other public reporting, which would harm our business and the trading price of our common stock.

We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act of 2002 and the rules and regulations of The Nasdaq Global Market. Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, we are required to perform system and process evaluation and testing of our internal control over financial reporting to allow our management to report on the effectiveness of our internal control over financial reporting in this Form 10-K.

Effective internal control over financial reporting is necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, is designed to prevent fraud. Any failure to implement required new or improved controls, or difficulties encountered in their implementation, could cause us to fail to meet our reporting obligations. In addition, any testing by us, as and when required, conducted in connection with Section 404 of the Sarbanes-Oxley Act, or Section 404, or any subsequent testing by our independent registered public accounting firm, as and when required, may reveal deficiencies in our internal control over financial reporting that are deemed to be significant deficiencies or material weaknesses or that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement. Inferior internal controls could also cause investors to lose confidence in our reported financial information, which could have a negative effect on the trading price of our common stock.

Pursuant to Section 404, we are required to furnish a report by our management on our internal control over financial reporting beginning with this Annual Report. However, while we remain an EGC, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. When we cease to be an emerging growth company, we will be required to incur substantial additional professional fees and internal costs to expand our accounting and finance functions in order to include such attestation report.

We may in the future discover weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our financial statements. Our internal control over financial reporting will not prevent or detect all error and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected. If we identify one or more material weaknesses in our internal controls, investors could lose confidence in the reliability of our financial statements, the market price of our stock could decline and we could be subject to sanctions or investigations by The Nasdaq Global Market, the SEC or other regulatory authorities.

We may become involved in securities class action litigation or shareholder derivative litigation that could divert management's attention and harm our business and insurance coverage may not be sufficient to cover all costs and damages.

In the past, securities class action or shareholder derivative litigation has often followed certain significant business transactions, such as the sale of a business division or announcement of a merger. This risk is especially relevant for us because biopharmaceutical companies have experienced significant stock price volatility in recent years. We may become involved in this type of litigation in the future, including litigation, if any, that may result in connection with the recently completed Merger. Litigation often is expensive and diverts management's attention and resources, which could adversely affect our business.

Provisions in our corporate charter documents and under Delaware law could make an acquisition of the Company, 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 by-laws may discourage, delay or prevent a merger, acquisition or other change in control of the Company that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares. These provisions also could 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 the 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 the board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize the board of directors to issue preferred stock without stockholder approval, which could be used to institute a shareholder rights plan, or so-called "poison pill," that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by the 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 by-laws.

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 the Company 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.

Our certificate of incorporation provides that the Court of Chancery of the State of Delaware will be the exclusive forum for substantially all disputes between the Company and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with the Company or our directors, officers, employees or stockholders.

Our certificate of incorporation provides that the Court of Chancery of the State of Delaware is the exclusive forum for any derivative action or proceeding brought on the Company's behalf, any action asserting a breach of fiduciary duty owed by our directors, officers, other employees or stockholders to the Company or our stockholders, any action asserting a claim against the Company arising pursuant to the Delaware General Corporation Law or as to which the Delaware General Corporation Law confers jurisdiction on the Court of Chancery of the State of Delaware, or any action asserting a claim arising pursuant to our certificate of incorporation or by-laws or governed by the internal affairs doctrine. This provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with the Company or our directors, officers, employees or stockholders, which may discourage such lawsuits against the Company and our directors, officers, employees or stockholders.

Alternatively, if a court were to find this provision in our certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could adversely affect our business and financial condition.

ITEM 1B. UNRESOLVED STAFF COMMENTS

None.

ITEM 2. PROPERTIES

We lease approximately 12,577 square feet of office space at 955 Massachusetts Avenue, 4th Floor, Cambridge, Massachusetts, which currently serves as our corporate headquarters. The lease expires on July 31, 2022, and we have the option to extend the term one time for an additional five-year period. The base monthly payment on the lease is approximately $68 thousand as of December 31, 2019, subject to specified annual increases of approximately 1.5% during the term of the lease and not including operating expenses, certain utilities, taxes and insurance for which we are responsible.

On November 11, 2019, we entered into a 7-year lease agreement for approximately 28,000 square feet of office space currently under construction in a building located at 61 North Beacon Street, Allston, Massachusetts. The lease will replace our current headquarters in 2020. Beginning in May 2020, the base monthly rent payment on the lease will be approximately $82 thousand, which is subject to scheduled annual increases for the term of the lease, plus certain operating expenses. We will incur construction costs of approximately $4.8 million to prepare the office space for our use, of which the landlord will reimburse $1.8 million. We are required to provide the landlord with a $1.1 million security deposit in the form of a letter of credit, which is classified as long-term restricted cash on our balance sheet as of December 31, 2019. We have the right to sublease the premises, subject to landlord consent and we have the right to renew the lease for an additional five years at the then prevailing effective market rental rate.

ITEM 3. LEGAL PROCEEDINGS

From time to time, we may become involved in legal proceedings arising in the ordinary course of our business. We are not currently subject to any material legal proceedings.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

PART II

ITEM 5. MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS AND ISSUER PURCHASES OF EQUITY SECURITIES

Market Information

Our common stock commenced trading on the Nasdaq Global Market under the symbol “ASNS” on November 16, 2017. Prior to that date, there was no public trading market for our common stock. On March 13, 2019, we completed a business combination in accordance with the terms of the Merger Agreement, by and among us, X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals, Inc.) and the Merger Sub, pursuant to which, among other matters, Merger Sub merged with and into X4 Therapeutics, Inc., with X4 Therapeutics, Inc. continuing as our wholly-owned subsidiary and the surviving corporation of the merger. Following the Merger, on March 13, 2019, we effected a 1-for-6 reverse stock split of our common stock and changed our name to “X4 Pharmaceuticals, Inc.” On March 13 2019, following the completion of the Merger, our common stock began trading on the Nasdaq Global Market under the symbol “XFOR”.

Holders of Our Common Stock

As of March 6, 2020, there were 52 holders of record of our common stock.

Dividend Policy

We have never declared or paid cash dividends on our common stock. We currently intend to retain all available funds and any future earnings to fund the development and expansion of our business and we do not anticipate paying any cash dividends in the foreseeable future. Any future determination to declare and pay dividends will be made at the discretion of our board of directors and will depend on then-existing conditions, including our results of operations, financial condition, contractual restrictions, capital requirements, business prospects and other factors our board of directors may deem relevant.

Recent Sales of Unregistered Securities

None.

Purchase of Equity Securities by the Issuer and Affiliated Purchasers

None.

ITEM 6. SELECTED FINANCIAL DATA

As a smaller reporting company, we are not required to provide disclosure for this Item.

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 the related notes and the other financial information included elsewhere in this Annual Report. Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this Annual Report, our actual results could differ materially from the results described in or implied by these forward-looking statements.

For the discussion of the financial condition and results of operations for the year ended December 31, 2018 compared to the year ended December 31, 2017, refer to "Management's Discussion and Analysis of Financial Condition and Results of Operations" *filed as Exhibit 99.1 to our Current Report on Form 8-K filed with the SEC on August 9, 2019.*

Overview

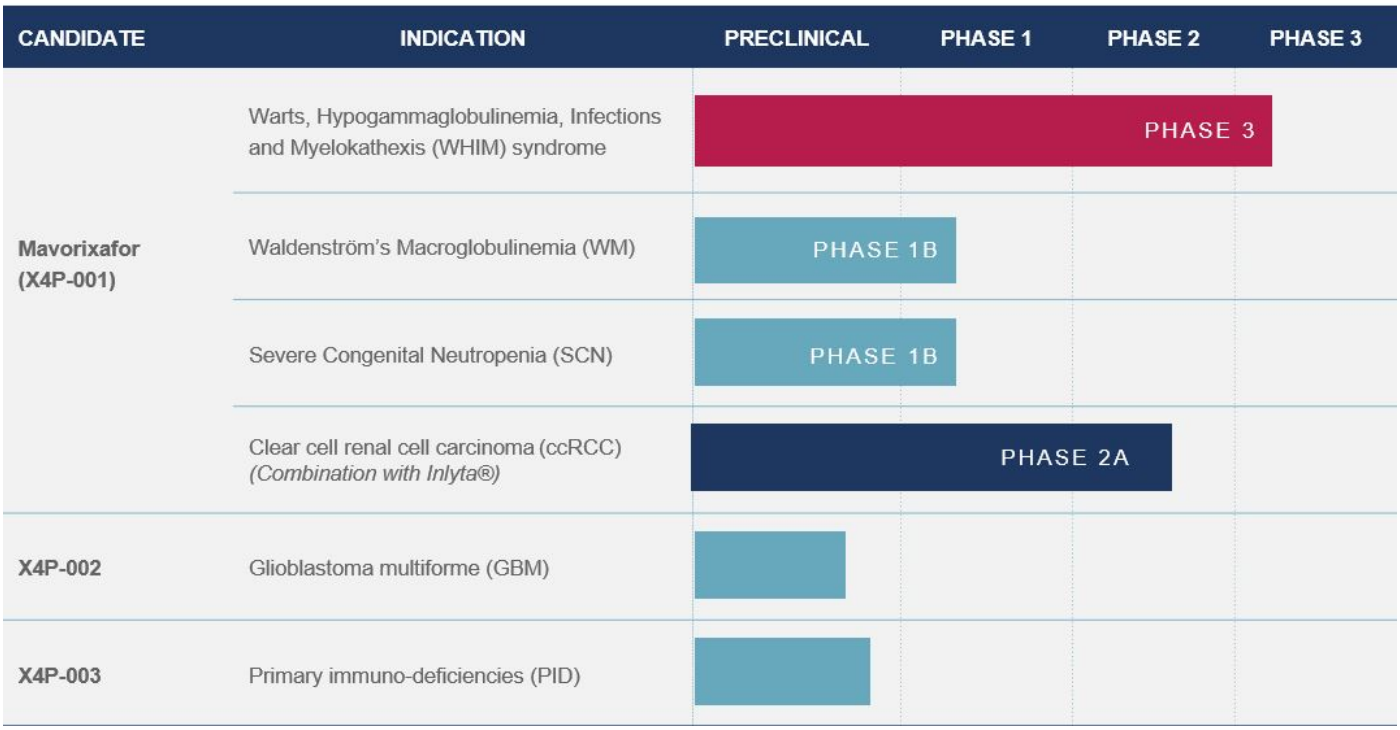
We are a clinical-stage biopharmaceutical company focused on the research, development and commercialization of novel therapeutics for the treatment of rare diseases. Our pipeline is comprised of potentially first-in-class, oral, small molecule antagonists of chemokine receptor CXCR4, which have the potential to treat a broad range of rare diseases, including primary immunodeficiencies, or PIs, and certain types of cancer. PIs are a group of more than 250 rare, chronic disorders in which flaws in the immune system cause increased susceptibility to infections and, in some cases, increased risk of cancers. Within this broad disease classification, a number of PIs are attributed to the improper trafficking of immune cells related to the CXCR4 receptor and its ligand CXCL12. CXCR4 is stimulated by its only chemokine ligand, CXCL12, and plays a key role in enabling the trafficking of immune cells and effectively monitoring the function of the immune system, or immunosurveillance. Overstimulation of the CXCL12/CXCR4 pathway leads to inhibition of the immune response, or immunosuppression.
Our lead product candidate, mavorixafor, is a potentially first-in-class, oral, allosteric antagonist of the CXCR4 receptor designed to correct the abnormal signaling caused by the receptor/ligand interaction and enable mobilization and trafficking of immune cells. Mavorixafor has completed a Phase 2 clinical trial in patients with Warts, Hypogammaglobulinemia, Infections, and Myelokathexis, or WHIM, syndrome, which is a rare, inherited primary immunodeficiency disease. In June 2019, we announced the initiation of 4WHIM, a pivotal, 52-week Phase 3 global clinical trial of mavorixafor for the treatment of patients with WHIM syndrome. We plan to report top-line data from this trial in the second half of 2021. In November 2019, the FDA granted Breakthrough Therapy Designation for mavorixafor for the treatment of adults with WHIM. We are also investigating mavorixafor in combination with axitinib (Inlyta®) in the Phase 2a portion of an open-label Phase 1/2 clinical trial in clear cell renal cell carcinoma, or ccRCC. In September 2019, we announced positive results from the Phase 2a portion of our open-label Phase 1/2 clinical trial of mavorixafor in combination with axitinib (Inlyta®) in patients with advanced ccRCC in combination with axitinib, an FDA-approved small molecule tyrosine kinase inhibitor. Future development and potential commercialization of mavorixafor in ccRCC and other possible immuno-oncology indications will be pursued only as part of a potential strategic collaboration.

In November 2019, we announced the initiation of a 14-day, proof-of-concept Phase 1b clinical trial of mavorixafor in another PI, severe congenital neutropenia, or SCN. In December 2019, we announced the initiation of a Phase 1b clinical trial of mavorixafor in Waldenström macroglobulinemia, or WM. We expect to report initial data from the SCN and WM trials in the second half of 2020.

We are also advancing two early stage candidates towards the clinic: X4P-003, a second-generation CXCR4 antagonist designed to have an enhanced pharmacokinetic profile relative to mavorixafor, potentially enabling improved patient compliance and ease of use; and X4P-002, a CXCR4 antagonist designed to cross blood-brain barrier and provide appropriate therapeutic exposures to treat brain cancers.

To date, we have not generated revenue from product sales and do not expect to generate significant revenue from the sale of our products in the foreseeable future. If our development efforts for our product candidates are successful and result in regulatory approval, we may generate revenue in the future from product sales. We cannot predict if, when, or to what extent we will generate revenue from the commercialization and sale of our product candidates. We may never succeed in obtaining regulatory approval for any of our product candidates.

Our Pipeline:



Results of Operations

Comparison of the Years Ended December 31, 2019 and 2018

The following table summarizes the results of our operations for the periods indicated:

	Year Ended December 31,		
	2019	2018	Change
	(in thousands)		
Operating expenses:			
Research and development	$ 30,163	$ 20,346	$ 9,817
General and administrative	17,640	8,739	8,901
Loss on transfer of nonfinancial assets	3,900	—	3,900
Total operating expenses	51,703	29,085	22,618
Loss from operations	(51,703)	(29,085)	(22,618)
Other income (expense):			
Interest income	1,197	236	961
Interest expense	(2,147)	(720)	(1,427)
Change in fair value of preferred stock warrant and derivative liabilities	(105)	(3,487)	3,382
Loss on extinguishment of debt	(566)	(229)	(337)
Other income	517	—	517
Total other income (expense), net	(1,104)	(4,200)	3,096
Net loss	$ (52,807)	$ (33,285)	$ (19,522)

Research and Development Expenses

Research and development expenses consist primarily of costs incurred in connection with the discovery and development of our product candidates, including employee salaries and related expenses, expenses incurred in connection with the preclinical and clinical development of our product candidates, including under agreements with third parties, such as consultants and contract research organizations, or CROs; the cost of manufacturing drug products for use in our preclinical studies and clinical trials, including under agreements with third parties, such as consultants and contract manufacturing organizations, or CMOs; facilities, depreciation and other expenses, which include direct or allocated expenses for rent and maintenance of facilities and insurance; costs related to compliance with regulatory requirements; and payments made under third-party licensing agreements. We expense research and development costs as incurred.

	Year Ended December 31,		
	2019	2018	Change
	(in thousands)		
Direct research and development expenses by product candidate:			
Mavorixafor (X4P-001)	$ 16,311	$ 10,625	$ 5,686
X4P-002	417	—	417
X4P-003	638	49	589
Unallocated expense	12,797	9,672	3,125
Total research and development expenses	$ 30,163	20,346	$ 9,817

Research and development expenses were $30.2 million for the year ended December 31, 2019 compared to $20.3 million for the year ended December 31, 2018, reflecting an increase of $9.8 million. The increase in research and development expenses for the year ended December 31, 2019 was primarily due to a $6.3 million increase in compensation expenses due to additional personnel within our manufacturing, regulatory, program management, and clinical operations functions as well as compensation expenses related to our research and development facility in Vienna, Austria, which we acquired in the Merger with Arsanis. These compensation expenses, the majority of which are not allocated to our programs, were partially offset by a decrease of $2.6 million in outside consulting costs as we replaced these outside costs with internal personnel. In addition, research and development expenses increased approximately $5.8 million due to direct expenses related primarily to our mavorixafor program, including the costs incurred in the current Phase 3 clinical development stage, and for outsourced services.

We expect that our research and development expenses, particularly for our mavorixafor programs, will increase over the next several years as we continue to conduct our Phase 3 pivotal trial of mavorixafor in patients with WHIM syndrome, and to conduct our Phase 1b clinical trials of mavorixafor in SCN and WM.

Research and development expenses related to our X4P-002 and X4P-003 programs were not significant in 2019 relative to our overall research and development expenses.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and related costs, including stock-based compensation, for personnel in executive, finance and administrative functions. General and administrative expenses also include direct and allocated facility-related costs as well as professional fees for legal, patent, consulting, investor and public relations, accounting, and audit services.

General and administrative expenses were $17.6 million for the year ended December 31, 2019 compared to $8.7 million for the year ended December 31, 2018, reflecting an increase of $8.9 million. The increase in general and administrative expenses for the year ended December 31, 2019 was primarily due to an increase in personnel-related costs due to an increase in head count to support the growth of our business, a significant increase in professional fees associated with becoming a public company, including higher accounting and legal expenses, an increase in insurance and compliance fees, and higher legal costs incurred in

connection with our intellectual property portfolio. We expect the growth of general and administrative expenses will slow significantly relative to prior years, but will continue to grow as we prepare for the commercial launch of mavorixafor.

Loss on Transfer of Nonfinancial Assets

During the year ended December 31, 2019, we entered into contractual arrangements with two third parties that transferred the rights to develop and commercialize the programs underlying in-process research and development, or IPR&D, intangible assets that we acquired in the merger with Arsanis. As a result of the transfer of control of the IPR&D projects to third parties, we recorded a charge of $3.9 million to *loss on transfer of nonfinancial assets* during 2019. The loss included the carrying value of the IPR&D intangible assets prior to the transfer of control, partially offset by non-refundable, upfront fees received from the third parties. In accordance with the contractual arrangements with these third parties, we are entitled to future fees that are contingent on the success of the third parties in advancing the applicable IPR&D projects toward commercialization. There was no such loss in the year ended December 31, 2018.

Other Income (Expense), Net

	Year Ended December 31,		
	2019	2018	Change
	(in thousands)		
Interest income	$ 1,197	$ 236	$ 961
Interest expense	(2,147)	(720)	(1,427)
Change in fair value of preferred stock warrant and derivative liabilities	(105)	(3,487)	3,382
Loss on extinguishment of debt	(566)	(229)	(337)
Other income	517	—	517
Total other income (expense), net	$ (1,104)	$ (4,200)	$ 3,096

The decrease in other income (expense), net, of $3.1 million for the year ended December 31, 2019 as compared to 2018 was primarily due to a decrease in losses related to the change in the fair value of our preferred stock warrant and derivative liabilities in the year ended December 31, 2019 as compared to the prior year. Upon the closing of the Merger in March 2019, the preferred stock warrants were converted to warrants for the purchase of shares of common stock and, therefore, the associated liability was transferred to equity and was no longer adjusted to fair value. In addition, the derivative liability was adjusted to zero and will no longer be adjusted to fair value following the Merger. Other income (expense) also decreased due to an increase in interest income, which was due to higher cash equivalent investments during the year ended December 31, 2019 as compared to the prior year. These decreases in other income (expense), net, were partially offset by a $1.4 million increase in interest expense as a result of our increased borrowings during the year ended December 31, 2019, primarily related to our Loan and Security Agreement, or the Hercules Loan Agreement, with Hercules Capital, Inc., or Hercules, and interest expense on loans with Österreichische Forschungsförderungsgesellschaft mbH, or FFG, acquired from Arsanis in March 2019.

Income Taxes

Since our inception, we have not recorded an income tax benefit for the net losses we have incurred or for our earned research and development tax credits, as we believe, based upon the weight of available evidence, that it is not more likely than not that these net operating loss carryforwards and tax credits will be realized as an offset future taxable income. Accordingly, we have recorded a full valuation allowance against our net deferred tax assets at each balance sheet date.

Liquidity and Capital Resources

Source of Liquidity

Since our inception, we have incurred significant operating losses and negative cash flows from our operations. We have not yet commercialized any products and we do not expect to generate revenue from sales of any products for several years, if at all. We expect that our research and development and general and administrative expenses will continue to increase and, as a result, we will need additional capital to fund our future operations, which we may raise through a combination of equity offerings, debt

financings, other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements.

We have funded our operations to date primarily with proceeds from sales of common stock, warrants and prefunded warrants for the purchase of our preferred stock and our common stock, sales of preferred stock, proceeds from the issuance of convertible debt and borrowings under loan and security agreements. In 2019, we completed a merger with Arsanis and acquired its$26.4 million of cash, cash equivalents and restricted cash. In addition, in 2019, we raised $139.4 million, net of offering costs, through public offerings of common stock, prefunded warrants to purchase shares of common stock, and accompanying warrants to purchase shares of common stock. In June 2019, we received $9.8 million in net proceeds as a result of refinancing our Hercules Loan Agreement, under which, subject to approval by Hercules, an additional $5.0 million is available for borrowing through December 15, 2020 and an additional term loan of $10.0 million is available through June 15, 2022. Principal payments under the Hercules Loan Agreement commence in February 2022.

As of December 31, 2019, we had cash and cash equivalents of $126.2 million and restricted cash of $1.9 million. We believe that our current liquidity will be sufficient to meet our projected operating, investing and debt service requirements for at least the next twelve months.

Cash Flows

The following table summarizes our cash flow activities for each of the periods presented:

	Year Ended December 31,		
	2019	2018	2017
	(in millions)		
Net loss	$ (52.8)	$ (33.3)	$ (22.0)
Adjustments to reconcile net loss to net cash used in operating activities	7.9	4.7	—
Changes in operating assets and liabilities	(3.2)	3.2	0.7
Net cash used in operating activities	(48.1)	(25.4)	(21.3)
Net cash provided by (used in) investing activities	27.2	—	(0.3)
Net cash provided by financing activities	140.7	6.9	33.3
Impact of foreign exchange on cash and restricted cash	(0.2)	—	—
Net increase (decrease) in cash, cash equivalents and restricted cash	119.6	(18.5)	11.7
Cash, cash equivalents and restricted cash, beginning of period	8.5	27.0	15.3
Cash, cash equivalents and restricted cash, end of period	$ 128.1	$ 8.5	$ 27.0

Operating Activities During the year ended December 31, 2019, net cash used in operating activities was $48.1 million, primarily resulting from our net losses of $52.8 million, adjusted for noncash expenses of $7.9 million and changes in our operating assets and liabilities of $3.2 million. Noncash expenses primarily include the derecognition of IPR&D intangible assets included within "loss on transfer of nonfinancial assets" in our consolidated statements of operations and comprehensive loss and stock-based compensation expense. The change in operating assets and liabilities was primarily due to an increase in accounts payable due to the growth in our operating activities.

Investing Activities During the year ended December 31, 2019, net cash provided by investing activities consisted primarily of $26.4 million of cash and restricted cash acquired in connection with our Merger. There was no comparable net cash provided by or used in investing activities during the same period in the prior year.

Financing Activities During the year ended December 31, 2019, net cash provided by financing activities was $140.7 million, consisting primarily of $139.4 million of net proceeds for the sale in April 2019 and November 2019 of our common stock, warrants and prefunded warrants and net proceeds of $9.8 million received in our 2019 Loan Agreement with Hercules, partially offset by $9.4 million for the settlement of our FFG loans. During the year ended December 31, 2018, net cash provided by financing activities was $6.9 million, consisting primarily of net proceeds of $4.4 million from the issuance of Series B convertible preferred stock and $10.0 million of proceeds from borrowings under the Hercules Loan Agreement, partially offset

by $6.4 million of repayments of borrowings under our SVB Loan Agreement and $1.1 million for the repurchase of Series Seed convertible preferred stock pursuant to a stock repurchase agreement we entered into in October 2017.

Loan and Security Agreements

Loan and Security Agreement with Silicon Valley Bank

In October 2016, we entered into a Loan and Security Agreement, or the SVB Loan Agreement, with Silicon Valley Bank, or SVB, which provided for certain term loans, including a term loan of up to $6.0 million, which we borrowed in June 2017. In October 2018, in connection with entering into the Hercules Loan Agreement (as described further below), we terminated the SVB Loan Agreement and repaid all amounts due under the SVB Loan Agreement, including unpaid principal of $4.3 million, a final payment of $0.3 million, a prepayment premium of $87 thousand and accrued interest of $23 thousand.

Loan and Security Agreement with Hercules Capital, Inc.

In October 2018, we entered into the Hercules Loan Agreement with Hercules. The Hercules Loan Agreement was amended in December 2018 and in June 2019. As of December 31, 2019, the Hercules Loan Agreement, as amended, provides for aggregate maximum borrowings of $35.0 million, of which $20.0 million is outstanding, an additional $5.0 million is available for borrowing through December 15, 2020 and, subject to approval by Hercules, an additional term loan of $10.0 million is available through June 15, 2022.

Borrowings under the Hercules Loan Agreement bear interest at a variable rate equal to the greater of (i) 8.75% or (ii) 8.75% plus *The Wall Street Journal* prime rate minus 6.0%. In an event of default, as defined in the Hercules Loan Agreement, and until such event is no longer continuing, the interest rate applicable to borrowings under the agreement would be increased by 4.0%. As of December 31, 2019, the interest rate applicable to borrowings was 8.75%.

Borrowings under the agreement are repayable in monthly interest-only payments through January 1, 2022, and in equal monthly payments of principal and accrued interest from February 1, 2022 until the maturity date of the loan, which is July 1, 2023. At our option, we may prepay all, but not less than all, of the outstanding borrowings, subject to a prepayment premium of up to 2.0% of the principal amount outstanding as of the date of repayment. In addition, the Hercules Loan Agreement provides for payments of (i) $0.8 million payable upon the earlier of November 1, 2021 or the repayment in full of all obligations under the agreement, and (ii) 4.0% of the aggregate principal amount of advances drawn under the Hercules Loan Agreement payable upon the earlier of maturity or the repayment in full of all obligations under the agreement.

Borrowings under the Hercules Loan Agreement are collateralized by substantially all of our personal property and other assets except for our intellectual property (but including rights to payment and proceeds from the sale, licensing or disposition of the intellectual property). Our obligations under the agreement are subject to acceleration upon occurrence of specified events of default, including payment default, insolvency and a material adverse change in our business, operations or financial or other conditions.

Under the agreement, we have agreed to affirmative and negative covenants to which we will remain subject until maturity or repayment of the loan in full. The covenants include (a) maintaining a minimum liquidity amount of the lesser of (i) 125% of the aggregate principal amount of outstanding borrowings under the Hercules Loan Agreement and (ii) 100% of our cash and cash equivalents (other than up to $2.5 million which may be held by an excluded subsidiary, as defined) in an account in which Hercules has a first priority security interest, as well as (b) restrictions on our ability to incur additional indebtedness, pay dividends, encumber our intellectual property, or engage in certain fundamental business transactions, such as mergers or acquisitions of other businesses. The Hercules Loan Agreement also contains a covenant that required us to repay our FFG loans on or prior to December 31, 2019, and we repaid the FFG loans in September 2019. We were in compliance with all covenants under the Hercules Loan Agreement as of December 31, 2019.

FFG Borrowings

Between September 2011 and March 2017, Arsanis GmbH, a subsidiary of Arsanis, entered into a series of funding agreements with FFG that provided for loans and grants to fund qualifying research and development expenditures of Arsanis GmbH on a project-by-project basis, as approved by FFG. On March 8, 2019, Arsanis entered into a settlement agreement, or the FFG Settlement Agreement, with FFG in respect of allegations that Arsanis breached certain reporting, performance and other obligations. Pursuant to the terms of the FFG Settlement Agreement, in exchange for FFG's waiver of all claims against Arsanis and Arsanis GmbH except for its claims for repayment of the loans and regular interest, including its waiver of claims for repayment of grants and interest exceeding regular interest, Arsanis GmbH agreed to repay the outstanding loan principal (plus

regular interest accrued thereon) on an accelerated payment schedule of three years instead of the original five years, with the final accelerated installment due and payable on June 30, 2021. The FFG Settlement Agreement also contained certain other restrictive covenants, including a requirement to maintain, as of April 30, 2019, a minimum cash balance equal to 70% of the outstanding principal amount of the loans in an account held with an Austrian bank, until all of the loans have been repaid and subject to other terms specified in the FFG Settlement Agreement. Amounts due under the FFG loans bore interest at varying fixed rates ranging from 0.75% to 2.0% per annum.

During the quarter ended September 30, 2019, we re-paid the full amount due on the FFG loan of approximately $6.5 million, and the FFG loans were retired. We recognized a loss on extinguishment of debt of $0.6 million related to the unamortized debt discount as of the date of retirement.

Funding Requirements

We expect our expenses to increase in connection with our ongoing activities, particularly as we advance the clinical trials of our product candidates in development. In addition, we expect to continue to incur additional costs operating as a public company. Because of the numerous risks and uncertainties associated with research, development and commercialization of pharmaceutical product candidates, we are unable to estimate the exact amount of our working capital requirements. Our future funding requirements will depend on and could increase significantly as a result of many factors, including:

- the scope, number, initiation, progress, timing, costs, design, duration, any potential delays, and results of clinical trials and nonclinical studies for our current or future product candidates, particularly our Phase 3 pivotal clinical trial of mavorixafor for the treatment of patients with WHIM syndrome, our Phase 1b clinical trial of mavorixafor in SCN and our Phase 1b clinical trial of mavorixafor in WM;
- the clinical development plans we establish for these product candidates;
- the number and characteristics of product candidates and programs that we develop or may in-license;
- the outcome, timing and cost of regulatory reviews, approvals or other actions to meet regulatory requirements established by the FDA and comparable foreign regulatory authorities, including the potential for the FDA or comparable foreign regulatory authorities to require that we perform more studies for our product candidates than those that we currently expect;
- our ability to obtain marketing approval for our product candidates;
- the cost of filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights covering our product candidates, including any such patent claims and intellectual property rights that we have licensed from Genzyme pursuant to the terms of our license agreement with Genzyme;
- our ability to maintain, expand and defend the scope of our intellectual property portfolio, including the cost of defending intellectual property disputes, including patent infringement actions brought by third parties against us or our product candidates;
- the cost and timing of completion of commercial-scale outsourced manufacturing activities with respect to our product candidates;
- our ability to establish and maintain licensing, collaboration or similar arrangements on favorable terms and whether and to what extent we retain development or commercialization responsibilities under any new licensing, collaboration or similar arrangement;
- the cost of establishing sales, marketing and distribution capabilities for any product candidates for which we may receive regulatory approval in regions where we choose to commercialize our products on our own;
- the success of any other business, product or technology that we acquire or in which we invest;
- the costs of acquiring, licensing or investing in businesses, product candidates and technologies;
- our need and ability to hire additional management and scientific and medical personnel;
- the costs to continue to operate as a public company, including the need to implement additional financial and reporting systems and other internal systems and infrastructure for our business;
- market acceptance of our product candidates, to the extent any are approved for commercial sale; and

- the effect of competing technological and market developments.

We expect that our existing cash and cash equivalents will be sufficient to fund our operating expenses, capital expenditure requirements and debt service payments for at least the next twelve months. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our capital resources sooner than we expect.

Until such time, if ever, as we can generate substantial product revenue, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, and marketing, distribution or licensing arrangements with third parties. In January 2019, we filed a universal shelf registration statement on Form S-3 with the SEC, which was declared effective in February 2019, pursuant to which we registered for sale up to $150 million of any combination of our common stock, preferred stock, debt securities, warrants and/or units from time to time and at prices and on terms that we may determine. Subsequently, in April 2019, we raised $79.3 million, net of offering costs, for the sale of common stock, prefunded warrants to purchase shares of common stock, and accompanying Class A warrants to purchase shares of our common stock. As of the date of this Annual Report, $12 million of securities remain available for issuance under this shelf registration statement. In August 2019, we filed a shelf registration statement on Form S-3 with the SEC, which was declared effective in August 2019, pursuant to which we registered for sale up to $150 million of any combination of our common stock, preferred stock, debt securities, warrants and/or units from time to time and at prices and on terms that we may determine. Subsequently, in November 2019, we raised $60.1 million, net of offering costs, for the sale of common stock, prefunded warrants to purchase shares of common stock or Class B warrants, and accompanying Class B warrants to purchase shares of our common stock. As of the date of this Annual Report, approximately $3.0 million of securities remain available for issuance under this shelf registration statement.

To the extent that we raise additional capital through future equity offerings or debt financings, the ownership interest of our stockholders may be materially diluted, and the terms of such securities could include liquidation or other preferences that adversely affect the rights of our stockholders. Debt financing and preferred equity financing, if available, may involve agreements that include restrictive covenants that limit our ability to take specified actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution 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 or other arrangements when needed, we may be required to delay, reduce or eliminate our product development efforts 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

As a smaller reporting company, we are not required to provide the disclosure required by Item 303(a)(5) of Regulation S-K.

Critical Accounting Policies and Significant Judgments and Estimates

Our consolidated financial statements are prepared in accordance with generally accepted accounting principles in the United States. The preparation of our consolidated financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, costs and expenses, and the disclosure of contingent assets and liabilities in our consolidated financial statements. We base our estimates on 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. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in Note 2 to our consolidated financial statements included elsewhere in this Annual Report, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our consolidated financial statements.

Accrued Research and Development Expenses. As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued research and development expenses. This process involves reviewing open contracts and

purchase orders, communicating with our applicable personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual costs. The majority of our service providers invoice us in arrears for services performed, on a pre-determined schedule or when contractual milestones are met; however, some require advance payments. We make estimates of our accrued expenses as of each balance sheet date in the consolidated financial statements based on facts and circumstances known to them at that time. We periodically confirm the accuracy of these estimates with the service providers and make adjustments, if necessary. Examples of estimated accrued research and development expenses include fees paid to:

- vendors in connection with preclinical development activities;
- CROs and investigative sites in connection with preclinical studies and clinical trials; and
- CMOs in connection with the production of preclinical and clinical trial materials.

We base the expense recorded related to external research and development on our estimates of the services received and efforts expended pursuant to quotes and contracts with multiple CMOs and CROs that supply, conduct and manage preclinical studies and clinical trials 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 expense. Payments under some of these contracts depend on factors such as the successful enrollment of patients and the completion of clinical trial milestones. 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 the estimate, we adjust the accrual or the amount of prepaid expenses 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 may result in reporting amounts that are too high or too low in any particular period. To date, there have not been any material adjustments to our prior estimates of accrued research and development expenses.

Stock-Based Compensation. We measure all stock-based awards granted to employees, directors and consultants based on the grant-date fair value of the award and recognized compensation expense, net of estimated forfeitures, over the requisite service period, which is generally the vesting period of the respective award. The stock-based awards that we have issued to date include a service-based vesting condition and the expense for these awards is recognized using the straight-line method. To date, we have not issued stock-based awards with performance-based vesting conditions.

The fair value of stock option grants is estimated on the date of grant using the Black-Scholes option-pricing model, which uses as inputs the fair value of our common stock and assumptions we make for the volatility of our common stock, the expected term of the stock options, the risk-free interest rate for a period that approximates the expected term of our stock options and their expected dividend yield. Prior to the closing of the Merger and the listing of our common stock on the Nasdaq Capital Market, our board of directors historically determined, as of the date of each option grant and with input from our management, the assistance of a third-party valuation specialist the estimated fair value of our common stock on the date of grant based on a number of objectives and subjective factors. Since the Merger and the listing of our common stock on the Nasdaq Capital Market, we have relied on the market price of our common stock to determine the fair value on the date of grant. As our common stock does not have a sufficient history of trading, we estimate our volatility based on the historical volatility of publicly traded peer companies. We estimate the expected term of our stock awards by utilizing the “simplified” method, which calculates the expected term based on weighted average midpoint of the award’s vesting and expiration dates. We determine the risk-free interest rate by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. We estimate that no dividends will be paid as we do not expect to pay cash dividends in the foreseeable future.

The assumptions underlying these valuations represent the best estimates of our management, which involve inherent uncertainties and the application of our judgment. As a result, if factors or expected outcomes change and we use significantly different assumptions or estimates, the resulting share-based compensation expense could be materially different.

Valuation of Preferred Stock Warrant Liability. In connection with our preferred stock financings prior to our merger with Arsanis in March 2019, we issued warrants to purchase shares of our preferred stock. We classified these warrants as liabilities on our consolidated balance sheet because they were deemed to be freestanding financial instruments that may have required us to transfer assets upon exercise. The warrant liability was initially recorded at fair value upon the date of issuance of each warrant

and is subsequently remeasured to fair value at each reporting date as a component of other income (expense), net in our consolidated statements of operations. Upon the closing of the Merger on March 13, 2019, all outstanding preferred stock warrants become exercisable for Arsanis common stock and, accordingly, the warrant liability as of March 13, 2019 was remeasured to fair value and reclassified to additional paid-in capital. As a result, following the closing of the Merger, we no longer recognize changes in the fair value of the warrant liability as other income (expense), net in our consolidated statement of operations and comprehensive loss.

To value the preferred stock warrants, we used various valuation methods, including the Monte Carlo method, the option-pricing method and the hybrid method, all of which incorporated assumptions and estimates. We assessed these assumptions and estimates on a quarterly basis as additional information impacting the assumptions is obtained. Estimates and assumptions impacting the fair value measurement included the fair value per share of the underlying shares of our Series A and Series B preferred stock, risk-free interest rate, expected dividend yield, expected volatility of the price of the underlying preferred stock, and the remaining contractual term of the warrants (except for the warrants that would be automatically exercised upon an initial public offering, in which case the remaining estimated term to automatic exercise was used). The most significant assumption in the Monte Carlo method, the option-pricing method and the hybrid method impacting the fair value of the preferred stock warrants was the fair value of our preferred stock as of each remeasurement date. We determined the fair value per share of the underlying preferred stock by taking into consideration the most recent sales of our preferred stock, results obtained from third-party valuations and additional factors that are deemed relevant.

Purchase Accounting. Business combinations are accounted for under the acquisition method, whereby the total purchase price of an acquisition is allocated to the underlying identifiable net assets, based on their respective estimated fair values as of the acquisition date. Application of the acquisition method is complex and can require significant judgment. For example, to properly account for a business combination, management must determine the following:

- Whether the entity acquired constitutes a business or a set of assets. Management considers whether the majority (i.e. 90%) of the value of the acquired entity is concentrated in one asset or set of similar assets.
- The identification of the acquirer for accounting purposes, which may differ from the legal acquirer. To make this determination, management considers which entity's stockholders own a substantial majority of the voting rights in the combined organization, (ii) the composition of the initial board of directors of the combined organization and (iii) the senior management team of the combined organization.
- Identification of transactions that must be accounted for separately from the acquired entity. For example, severance and retention bonuses which were arranged for the benefit of the acquirer.
- The fair value of consideration transferred to effect the acquisition, including the value of shares of common stock issued and contingent consideration transferred;
- The accounting for equity compensation arrangement acquired, including the fair value of replacement awards and whether the value of these awards is ascribed to compensation for past services, which is included in the consideration transferred to effect the acquisition, or for future services, which is accounted for prospectively as stock-based compensation expense
- The fair value of assets acquired and liabilities assumed, which requires management’s judgment and often involves the use of significant estimates and assumptions, including assumptions with respect to future cash inflows and outflows, probabilities of success, discount rates, and asset lives, among other items.

Lease Accounting. We adopted the new lease standard effective January 1, 2019 as further described in Note 2 to the consolidated financial statements included in this Annual Report on Form 10-K. The new standard requires that all leases, with the exception of leases with a remaining term of less than 12 months, are accounted for as a right-of-use asset with an associated lease liability on the consolidated balance sheet. Management must apply judgment in the application of the new lease standard in the determination of the following:

- Whether a contract contains a lease. Management considers whether the contract conveys the right to control an asset for a period of time in excess of one year. Management also considers whether the counterparty has the right to substitute the asset without management's consent, in which case the contract would generally not contain a lease.
- The term of the lease, which includes a determination of the probability that management will exercise its option to extend the lease.
- The discount rate applied to the lease liability, which in most cases is not a rate that is implicit and readily determinable within the lease. In this case, management calculates its incremental collateralized borrowing rate by reference to recent third-party borrowings, such as observed in its Hercules loan agreement, as adjusts this rate to match the term of the lease.

- The commencement date of the lease for accounting purposes, which occurs when the lessor provides management with unfettered access to the leased asset. Management also considers the condition of the leased asset at the inception of the lease agreement and whether unreimbursed costs incurred by management to enhance or construct the leased asset represent prepaid rent and are part of the right-of-use asset or represent leasehold improvements. Management considers the nature and significance of the improvements to the leased asset and whether these improvements were required by the lessor, which indicates that the improvements relate to the lessor's asset, or were at management's election, which indicates that the costs represent leasehold improvements.

Emerging Growth Company Status

The JOBS Act permits an “emerging growth company” such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have irrevocably elected to “opt out” of this provision and, as a result, we will comply with new or revised accounting standards when they are required to be adopted by public companies that are not emerging growth companies.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

Recently Issued Accounting Pronouncements

A description of recently issued accounting pronouncements that may potentially impact our financial position and results of operations is disclosed in Note 2 to our consolidated financial statements appearing at the end of this Annual Report on Form 10-K.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

As a smaller reporting company, we are not required to provide disclosure for this Item.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

The financial statements required to be filed pursuant to this Item 8 are appended to this report. An index of those financial statements is found in Item 15 of Part IV of this Annual Report on Form 10-K.

ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURES

None.

ITEM 9A. CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

We maintain “disclosure controls and procedures” as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, that are designed to ensure that information required to be disclosed in the reports we file and submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by us in the reports we file or submit under the Exchange Act is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosure. In designing and evaluating our disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the benefits of possible controls and procedures relative to their costs.

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, who serve as our principal executive officer and principal financial officer, respectively, has evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2019. Based on such evaluation, our Chief Executive Officer and Chief Financial Officer have concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of such date.

Management's Report on Internal Control Over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting for the company. Internal control over financial reporting is defined in Rule 13a-15(f) or 15d-15(f) promulgated under the Exchange Act as a process designed by, or under the supervision of, the company's principal executive and principal financial officers and effected by the company's board of directors, management and other personnel, 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 and includes those policies and procedures that:

- pertain to the maintenance of records that in reasonable detail accurately and fairly reflect the transactions and dispositions of the assets of the company;
- 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
- 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. 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.

Our management is responsible for establishing and maintaining an adequate system of internal control over financial reporting, as defined in the Exchange Act Rule 13a-15(f). Management conducted an assessment of our internal control over financial reporting based on the framework established in 2013 by the Committee of Sponsoring Organizations of the Treadway Commission in *Internal Control-Integrated Framework (2013)*. Based on the assessment, management concluded that, as of December 31, 2019, our internal control over financial reporting was effective.

This Annual Report does not include an attestation report of our registered public accounting firm regarding internal control over financial reporting due to an exemption established by the JOBS Act for "emerging growth companies".

Changes in Internal Control over Financial Reporting

There has been no significant change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the quarter ended December 31, 2019 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B. OTHER INFORMATION

None.

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

The information required by this Item is incorporated by reference to the information set forth in the sections titled "Proposal 1-Election of Directors," "Information About our Executive Officers," and "Information Regarding the Board of Directors and Corporate Governance" and "Delinquent Section 16(a) Reports" in our 2020 Proxy Statement.

ITEM 11. EXECUTIVE COMPENSATION

The information required by this Item is incorporated by reference to the information set forth in the sections titled "Executive Compensation" in our 2020 Proxy Statement.

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

The information required by this Item is incorporated by reference to the information set forth in the section titled "Security Ownership of Certain Beneficial Owners and Management" and "Equity Compensation Plan Information" in our 2020 Proxy Statement.

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

The information required by this Item is incorporated by reference to the information set forth in the section titled "Transactions with Related Persons and Indemnification" and "Information regarding the Board of Directors and Corporate Governance" in our 2020 Proxy Statement.

ITEM 14. PRINCIPAL ACCOUNTANT FEES AND SERVICES

The information required by this Item is incorporated by reference to the information set forth in the section titled "Principal Accountant Fees and Services" contained in our 2020 Proxy Statement.

PART IV

ITEM 15. EXHIBITS AND FINANCIAL STATEMENT SCHEDULES

(1) Financial Statements

The following documents are included on pages F-1 through F-39 attached hereto and are filed as part of this Annual Report.

(2) Financial Statement Schedules:

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

(3) Exhibits.

Exhibit No.	Exhibit Description	Form	Exhibit	Date	Se File/ Ref No.
2.1	Agreement and Plan of Merger, dated November 26, 2018, by and among the Company, Artemis AC Corp. and X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals, Inc.)	8-K	2.1	11/27/2018	001-38295
2.2	First Amendment to Agreement and Plan of Merger, dated December 20, 2018, by and among the Company, Artemis AC Corp. and X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals, Inc.)	8-K	2.1	12/20/2018	001-38295
2.3	Second Amendment to Agreement and Plan of Merger, dated March 8, 2019, by and among the Company, Artemis AC Corp. and X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals, Inc.)	8-K	2.1	3/8/2019	001-38295
3.1	Restated Certificate of Incorporation, filed with the Secretary of State of the State of Delaware on November 20, 2017.	8-K	3.1	11/20/2017	001-38295
3.2	Certificate of Amendment (Reverse Stock Split) to the Restated Certificate of Incorporation of the Company	8-K	3.1	3/13/2019	001-38295
3.3	Certificate of Amendment (Name Change) to the Restated Certificate of Incorporation of the Company	8-K	3.2	3/13/2019	001-38295
3.4	Amended and Restated By-laws of the Company	8-K	3.2	11/20/2017	001-38295
4.1	Form of Common Stock Certificate	8-K	4.1	3/13/2019	001-38295
4.2	Form of Warrant to Purchase Common Stock of the Company (formerly Series A Preferred Stock of X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals, Inc.)) issued to Silicon Valley Bank and Life Science Loans, LLC.	8-K	4.2	3/13/2019	001-38295
4.3	Form of Warrant to Purchase Common Stock of the Company (formerly Series A Preferred Stock of X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals, Inc.)) issued to Maxim Partners LLC.	8-K	4.3	3/13/2019	001-38295
4.4	Form of Warrant to Purchase Common Stock of the Company (formerly Series B Preferred Stock of X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals, Inc.)).	8-K	4.4	3/13/2019	001-38295

4.5	Form of Warrant to Purchase Common Stock of the Company (formerly Series B Preferred Stock of X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals, Inc.)) issued to Hercules Capital, Inc.	8-K	4.5	3/13/2019	001-38295
4.6	Warrant Modification Agreement, dated as of December 11, 2018, with Hercules Capital, Inc.	8-K	4.6	3/13/2019	001-38295
4.7	Form of Prefunded Warrant.	8-K	4.1	04/12/2019	001-38295
4.8	Form of Class A Warrant	8-K	4.2	04/12/2019	001-38295
4.9	Form of Prefunded Warrant	8-K	4.1	11/29/2019	001-38295
4.10	Form of Class B Warrant	8-K	4.2	11/29/2019	001-38295
4.11	Share Purchase Agreement, dated as of November 15, 2017, by and between the Company and New Enterprise Associates 16, L.P.	10-K	10.37	3/9/2018	001-38295
4.12*	Description of Registered Securities				001-38295
10.1@	2015 Employee, Director and Consultant Equity Incentive Plan, as amended.	8-K	10.1.1	3/13/2019	001-38295
10.2@	Form of Stock Option Agreement under the 2015 Employee, Director and Consultant Equity Incentive Plan, as amended.	8-K	10.1.2	4/2/2019	001-38295
10.3@	Form of Restricted Stock Unit Agreement under the 2015 Employee, Director and Consultant Equity Incentive Plan, as amended	8-K	10.6	6/17/2019	001-38295
10.4@	2017 Equity Incentive Plan	S-1	10.7	10/20/2017	001-38295
10.5@	Form of Incentive Stock Option Agreement under the 2017 Equity Incentive Plan	S-1	10.8	10/20/2017	001-38295
10.6@	Form of Nonstatutory Stock Option Agreement under the 2017 Equity Incentive Plan	S-1	10.9	10/20/2017	001-38295
10.7@	Form of Restricted Stock Agreement under the 2017 Equity Incentive Plan	8-K	10.6	11/27/2018	001-38295
10.8@	Form of Restricted Stock Unit Agreement under the 2017 Equity Incentive Plan	8-K	10.5	6/19/2019	001-38295
10.9@	2017 Employee Stock Purchase Plan	S-1	10.10	10/20/2017	001-38295
10.10@	X4 Pharmaceuticals, Inc. 2019 Inducement Equity Incentive Plan	8-K	10.1	6/17/2019	001-38295
10.11@	Form of Stock Option Agreement under the 2019 Inducement Equity Incentive Plan	8-K	10.2	6/17/2019	001-38295
10.12@	Form of Restricted Stock Agreement under the 2019 Inducement Equity Incentive Plan	8-K	10.3	6/17/2019	001-38295
10.13@	Form of Restricted Stock Unit Agreement under the 2019 Inducement Equity Incentive Plan	8-K	10.4	6/17/2019	001-38295
10.14@	Form of Indemnification Agreement (for directors and executive officers)	S-1/A	10.36	11/06/2017	001-38295
10.15@	Director Compensation Policy	8-K	10.2	3/13/2019	001-38295
10.16@	Amended and Restated Executive Employment Agreement, dated as of March 13, 2019, by and between the Company and Paula Ragan, Ph.D.	8-K	10.3	3/13/2019	001-38295
10.17@*	Amendment to Amended and Restated Executive Employment Agreement, dated as of March 13, 2019, dated February 13, 2020 by and between the Company and Paula Ragan, Ph.D.				
10.18@	Amended and Restated Executive Employment Agreement, dated as of March 13, 2019, by and between the Company and Adam S. Mostafa.	8-K	10.4	3/13/2019	001-38295
10.19@*	Amendment to Amended and Restated Executive Employment Agreement, dated as of February 24, 2020 by and between the Company and Adam S. Mostafa				

10.20@*	Executive Employment Agreement, dated as of April 16, 2019, by and between the Company and E. Lynne Kelley				
10.21@*	Amendment to Executive Employment Agreement, dated as of March 5, 2020, by and between the Company and E. Lynne Kelley				
10.22@*	Executive Employment Agreement, dated as of September 25, 2019, by and between the Company and Derek Meisner				
10.23@*	Amendment to Executive Employment Agreement, dated as of February 24, 2020, by and between the Company and Derek Meisner				
10.24#	License Agreement, dated as of July 10, 2014, by and between X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals, LLC) and Genzyme Corp., a Sanofi company.	8-K	10.5#	3/13/2019	001-38295
10.25#	Amendment No. 1 to License Agreement, dated as of October 23, 2014, by and between X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals, Inc.) and Genzyme Corporation, a Sanofi company.	8-K/A	10.6#	5/13/2019	001-38295
10.26#	License Agreement, dated as of December 13, 2016, by and between X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals, Inc.) and Georgetown University.	8-K/A	10.7#	5/13/2019	001-38295
10.27#	Exclusive License Agreement, dated as of December 23, 2016, by and between X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals, Inc.) and Beth Israel Deaconess Medical Center.	8-K/A	10.8#	5/13/2019	001-38295
10.28	Loan and Security Agreement, dated as of October 19, 2018, by and between X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals, Inc.) and Hercules Capital, Inc.	8-K	10.9	3/13/2019	001-38295
10.29	Amendment No. 1 to Loan and Security Agreement, dated as of December 11, 2018, by and between X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals, Inc.) and Hercules Capital, Inc.	8-K	10.10	3/13/2019	001-38295
10.30	Amended and Restated Loan and Security Agreement, dated as of June 27, 2019, by and among X4 Pharmaceuticals, Inc., X4 Therapeutics, Inc., Hercules Capital, Inc. and Hercules Capital Funding Trust 2019-1.	8-K	10.1	6/28/2019	001-38295
10.31	Lease, dated as of January 20, 2017, by and between X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals, Inc.) and Brickman 955 Massachusetts LLC.	8-K	10.11	3/13/2019	001-38295
10.32*	Lease, dated as of November 11, 2019, by and between X4 Pharmaceuticals Inc. and Beacon North Village, LLC.	8-K			
10.33	Lease Termination Agreement, dated February 26, 2019, by and between Arsanis Biosciences GmbH and Wüstenrot Marxbox GmbH & Co. OG (as successor-in-interest to Marxbox Bauprojekt GmbH & Co. OG), as amended (English translation)	8-K	10.1	3/1/2019	001-38295
10.34	Settlement Agreement, dated as of March 8, 2019, by and among X4 Pharmaceuticals, Inc. (formerly Arsanis, Inc.), Artemis AC Corp., X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals, Inc.), Arsanis Biosciences GmbH and Österreichische Forschungsförderungsgesellschaft GmbH.	8-K	10.1	4/11/2019	001-38295
10.35*	Master Services Agreement, dated September 10, 2015, by and between X4 Pharmaceuticals Inc. and Mayne Pharma Inc. (formerly known as Metrics, Inc.).				
10.36*	Amendment No. 1 to Master Services Agreement, dated August 25, 2017, by and between X4 Pharmaceuticals Inc. and Mayne Pharma Inc. (formerly known as Metrics, Inc.)				
10.37*	Amendment No. 2 to Master Services Agreement, dated February 28, 2020, by and between X4 Pharmaceuticals Inc. and Mayne Pharma Inc.				
10.38*	Master Services Agreement, dated February 19, 2016, by and between X4 Pharmaceuticals Inc. and Aptuit (Oxford) Limited				

10.39*	Amendment No. 1 to Master Services Agreement, dated February 19, 2016, by and between X4 Pharmaceuticals Inc. and Aptuit (Oxford) Limited
21.1*	List of Subsidiaries
23.1*	Consent of PricewaterhouseCoopers LLP, Independent Registered Public Accounting Firm
31.1*	Certification of the Chief Executive Officer, as required by Section 302 of the Sarbanes-Oxley Act of 2002
31.2*	Certification of the Chief Financial Officer, as required by Section 302 of the Sarbanes-Oxley Act of 2002
32.1**	Certification of the Chief Executive Officer, as required by Section 906 of the Sarbanes-Oxley Act of 2002
32.2**	Certification of the Chief Financial Officer, as required by Section 906 of the Sarbanes-Oxley Act of 2002
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	SXRL 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

** Furnished and not filed herewith

Certain confidential portions of this Exhibit were omitted by means of marking such portions with brackets ("[***]") because the identified confidential portions (i) are not material and (ii) would be competitively harmful if publicly disclosed

@ Indicates management contract or compensatory plan

ITEM 16. FORM 10-K SUMMARY

None.

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.

X4 PHARMACEUTICALS, INC.

Date: March 12, 2020

By: /s/ Paula Ragan
Paula Ragan, Ph D.
President and Chief Executive Officer

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

Signature	Title	Date
/s/ Paula Ragan Paula Ragan, Ph.D.	President, Chief Executive Officer and Director *(Principal Executive Officer)*	March 12, 2020
/s/ Adam S. Mostafa Adam S. Mostafa	Chief Financial Officer and Treasurer *(Principal Financial Officer and Principal Accounting Officer)*	March 12, 2020
/s/ Michael S. Wyzga Michael S. Wyzga	Chairman of the Board of Directors	March 12, 2020
/s/ William Aliski William E. Aliski	Director	March 12, 2020
/s/ Gary J. Bridger Gary J. Bridger, Ph.D.	Director	March 12, 2020
/s/ David McGirr David McGirr	Director	March 12, 2020
/s/ René Russo René Russo, Pharm D	Director	March 12, 2020
/s/ Murray W. Stewart Murray W. Stewart, M.D.	Director	March 12, 2020

INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Board of Directors and Stockholders of X4 Pharmaceuticals, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of X4 Pharmaceuticals, Inc. and its subsidiaries (the "Company") as of December 31, 2019 and 2018, and the related consolidated statements of operations and comprehensive loss, of convertible preferred stock, redeemable common stock and stockholders' equity (deficit) and of cash flows for each of the three years in the period ended December 31, 2019, including the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company as of 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 accounting principles generally accepted in the United States of America.

Change in Accounting Principle

As discussed in Note 2 to the consolidated financial statements, the Company changed the manner in which it accounts for leases in 2019.

Basis for Opinion

These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's consolidated financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (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 of these consolidated financial statements in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audits to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the consolidated 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 consolidated 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 consolidated financial statements. We believe that our audits provide a reasonable basis for our opinion.

Emphasis of Matter

As discussed in Note 1 to the consolidated financial statements, the Company will require additional financing to fund future operations. Management's evaluation of the events and conditions and plans to mitigate this matter are also described in Note 1.

/s/ PricewaterhouseCoopers LLP
Boston, Massachusetts
March 12, 2020

We have served as the Company's auditor since 2016.

X4 PHARMACEUTICALS INC.
CONSOLIDATED BALANCE SHEETS
(In thousands, except share and per share amounts)

	December 31, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 126,184	$ 8,134
Research and development incentive receivable	1,998	—
Prepaid expenses and other current assets	1,096	1,205
Total current assets	129,278	9,339
Property and equipment, net	403	241
Goodwill	27,109	—
Right-of-use assets	1,959	—
Other assets	1,949	364
Total assets	$ 160,698	$ 9,944
Liabilities, Convertible Preferred Stock, Redeemable Common Stock and Stockholders' Equity (Deficit)		
Current liabilities:		
Accounts payable	$ 2,088	$ 2,969
Accrued expenses	6,461	3,251
Current portion of lease liability	898	—
Current portion of long-term debt, net of discount	—	1,687
Total current liabilities	9,447	7,907
Preferred stock warrant liability	—	4,947
Long-term debt, including accretion, net of discount and current portion	20,097	8,145
Deferred rent	—	417
Lease liabilities	1,918	—
Other liabilities	16	205
Total liabilities	31,478	21,621
Commitments and contingencies (Note 10)		
Convertible preferred stock, $0.001 par value; 10,000,000 and 59,413,523 shares authorized as of December 31, 2019 and December 31, 2018, respectively; 0 and 40,079,567 shares issued and outstanding as of December 31, 2019 and December 31, 2018, respectively	—	64,675
Redeemable common stock, $0.001 par value; 0 and 107,371 shares issued and outstanding as of December 31, 2019 and December 31, 2018, respectively	—	734
Stockholders' equity (deficit):		
Common stock, $0.001 par value. 33,333,333 and 11,070,776 shares authorized as of December 31, 2019 and December 31, 2018, respectively; 16,128,862 and 351,652 shares issued and outstanding as of December 31, 2019 and December 31, 2018, respectively	16	—
Additional paid-in capital	261,367	2,151
Accumulated other comprehensive income (loss)	(119)	—
Accumulated deficit	(132,044)	(79,237)
Total stockholders' equity (deficit)	129,220	(77,086)
Total liabilities, convertible preferred stock, redeemable common stock and stockholders' equity (deficit)	$ 160,698	$ 9,944

The accompanying notes are an integral part of these consolidated financial statements

X4 PHARMACEUTICALS, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(In thousands, except per share amounts)

	Year Ended December 31,		
	2019	2018	2017
Operating expenses:			
Research and development	$ 30,163	$ 20,346	$ 17,066
General and administrative	17,640	8,739	5,181
Loss on transfer of nonfinancial assets	3,900	—	—
Total operating expenses	51,703	29,085	22,247
Loss from operations	(51,703)	(29,085)	(22,247)
Other income (expense):			
Interest income	1,197	236	64
Interest expense	(2,147)	(720)	(490)
Change in fair value of preferred stock warrant liability	(288)	(3,398)	1,360
Change in fair value of derivative liability	183	(89)	(94)
Loss on preferred stock repurchase liability	—	—	(587)
Loss on extinguishment of debt	(566)	(229)	—
Other income	517	—	—
Total other income (expense), net	(1,104)	(4,200)	253
Net loss	(52,807)	(33,285)	(21,994)
Accruing dividends on Series A convertible preferred stock	(592)	(3,000)	(3,000)
Adjustment to accumulated deficit in connection with repurchase of Series Seed convertible preferred stock	—	(22)	—
Net loss attributable to common stockholders	$ (53,399)	$ (36,307)	$ (24,994)
Net loss per share attributable to common stockholders—basic and diluted	$ (4.63)	$ (79.15)	$ (54.58)
Weighted average shares of common stock outstanding—basic and diluted	11,530	459	458
Net loss	$ (52,807)	$ (33,285)	(21,994)
Currency translation adjustments	(119)	—	—
Total comprehensive loss	$ (52,926)	$ (33,285)	$ (21,994)

The accompanying notes are an integral part of these consolidated financial statements.

X4 PHARMACEUTICALS, INC.

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

(In thousands, except share amounts)

	Series Seed, A and B Convertible Preferred		Redeemable Common Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' (Deficit) Equity
	Shares	Amount	Shares	Amount	Shares	Amount				
Balance at December 31, 2016	**22,061,973**	**$ 34,307**	**107,371**	**$ 734**	**345,856**	**$ —**	**$ 884**	**$ —**	**$ (23,936)**	**$ (23,052)**
Issuance costs of convertible preferred stock, net of issuance costs of $2.6 million	15,956,995	26,596								**—**
Exercise of stock options					4,751	—	9			**9**
Stock-based compensation expense							492			**492**
Net loss									(21,994)	**(21,994)**
Balance at December 31, 2017	**38,018,968**	**60,903**	**107,371**	**734**	**350,607**	**—**	**1,385**	**—**	**(45,930)**	**(44,545)**
Repurchase of Series Seed convertible preferred stock, net of issuance costs of $11	(598,975)	(517)							(22)	**(22)**
Issuance costs of convertible preferred stock, net of issuance costs of $539	2,659,574	4,289								**—**
Exercise of stock options					1,045		7			**7**
Stock-based compensation expense							759			**759**
Net loss									(33,285)	**(33,285)**
Balance at December 31, 2018	**40,079,567**	**64,675**	**107,371**	**734**	**351,652**	**—**	**2,151**	**—**	**(79,237)**	**(77,086)**
Conversion of redeemable common stock into common stock			(107,371)	(734)	107,364	—	734			**734**
Conversion of convertible preferred shares into common stock	(40,079,567)	(64,675)			3,808,430	4	64,671			**64,675**
Exchange of common stock in connection with Merger					2,440,582	2	45,539			**45,541**
Fair value of replacement equity awards							817			**817**
Reclassification of warrant liability to permanent equity							5,235			**5,235**
Issuance of common stock and prefunded warrants for the purchase of common stock, net of issuance costs of $11.4 million					9,336,667	9	139,379			**139,388**
Exercise of stock options					50,321	1	344			**345**
Exercise of warrants					33,846	—	447			**447**
Stock-based compensation expense							2,050			**2,050**
Foreign currency translation adjustment								(119)		**(119)**
Net loss									(52,807)	**(52,807)**
Balance at December 31, 2019	**—**	**—**	**—**	**—**	**16,128,862**	**$ 16**	**$ 261,367**	**$ (119)**	**$ (132,044)**	**$ 129,220**

The accompanying notes are a integral part of these consolidated financial statements

X4 PHARMACEUTICALS, INC.

CONSOLIDATED STATEMENTS OF CASH FLOWS
(In thousands)

	Year Ended December 31,		
	2019	**2018**	**2017**
Cash flows from operating activities:			
Net loss	$ (52,807)	$ (33,285)	$ (21,994)
Adjustments to reconcile net loss to net cash used in operating activities:			
Stock-based compensation expense	2,050	759	492
Depreciation and amortization expense	103	103	71
Loss on transfer of non-financial assets	3,900	—	—
Non-cash lease expense	569	—	—
Accretion of debt discount	695	152	157
Loss on extinguishment of debt	566	229	—
Loss on preferred stock repurchase liability	—	—	587
Change in fair value of preferred stock warrant and derivative liability	105	3,487	(1,266)
Changes in operating assets and liabilities:			
Prepaid expenses, other current assets and research & development incentive receivable	109	279	402
Accounts payable	(2,752)	1,307	(1,169)
Accrued expenses	160	1,549	1,409
Lease liabilities	(753)	—	—
Net cash used in operating activities	(48,055)	(25,420)	(21,311)
Cash flows from investing activities:			
Cash, cash equivalents and restricted cash acquired in connection with the Merger	26,406	—	—
Proceeds from transfer of non-financial assets	1,000	—	—
Purchase of property, equipment and intangible assets	(174)	—	(378)
Net cash provided by (used in) investing activities	27,232	—	(378)
Cash flows from financing activities:			
Proceeds from exercise of stock options and warrants	792	7	9
Proceeds from borrowings under loan and security agreements, net of issuance costs	9,849	9,908	6,000
Proceeds from issuance of Series B convertible preferred stock, net of issuance costs	—	4,461	27,413
Repurchase of Series Seed convertible preferred stock	—	(1,126)	—
Repayments of borrowings under loan and security agreement	(9,368)	(6,380)	—
Proceeds from sale of common stock and warrants, net of issuance costs	139,388	—	—
Net cash provided by financing activities	140,661	6,870	33,422
Effect of exchange rate changes on cash, cash equivalents and restricted cash	(250)	—	—
Net increase (decrease) in cash, cash equivalents and restricted cash	119,588	(18,550)	11,733
Cash, cash equivalents and restricted cash at beginning of period	8,498	27,048	15,315
Cash, cash equivalents and restricted cash at end of period	$ 128,086	$ 8,498	$ 27,048
Supplemental disclosure of cash flow information			
Cash paid for interest	$ 1,284	$ 540	$ 282
Supplemental disclosure of non-cash investing and financing activities:			
Purchase of property, equipment and intangible assets included in accounts payable	$ 40	$ —	$ —
Issuance costs related to sale of common stock and warrants, not yet paid	$ 775		
Conversion of convertible preferred stock into common stock	$ 64,675	$ —	$ —
Conversion of redeemable common stock into common stock	$ 734	$ —	$ —
Conversion of convertible preferred stock warrants into common stock warrants	$ 5,235	$ —	$ —
Fair value of net assets acquired in the Merger in exchange for common shares, excluding cash acquired	$ 19,952	$ —	$ —
Initial fair value of derivative liability in connection with loan and security agreement	$ —	$ 18	$ —
Issuance of warrants in connection with Series B convertible preferred stock financing	$ —	$ 172	$ 817
Issuance of warrants in connection with loan and security agreement	$ —	$ 132	$ —

The accompanying notes are an integral part of these consolidated financial statements.

1. Nature of the Business and Basis of Presentation

X4 Pharmaceuticals, Inc. (formerly Arsanis, Inc.), together with its subsidiaries (the "Company"), is a clinical-stage biotechnology company focused on the research, development and commercialization of novel therapeutics for the treatment of rare diseases. The Company's lead product candidate, mavorixafor (X4P-001), is a potential first-in-class, once-daily, oral inhibitor of CXCR4 and is currently in a Phase 3 clinical trial for the treatment of WHIM syndrome, a rare, inherited, primary immunodeficiency disease caused by genetic mutations in the CXCR4 receptor gene. The Company is headquartered in Cambridge, Massachusetts.

The Company is subject to risks and uncertainties common to pre-commercialization companies in the biotechnology industry, including, but not limited to, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with governmental regulations and the ability to secure additional capital to fund operations. Drug candidates currently under development will require extensive preclinical and clinical testing and regulatory approval prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel, and infrastructure and extensive compliance-reporting capabilities. Even if the Company's drug development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

Merger with Arsanis

On November 26, 2018, Arsanis, Inc., a publicly held Delaware corporation ("Arsanis"), Artemis AC Corp., a Delaware corporation and a wholly-owned subsidiary of Arsanis ("Merger Sub"), and X4 Therapeutics, Inc. ("X4") entered into an Agreement and Plan of Merger, as amended on December 20, 2018 and March 8, 2019 (the "Merger Agreement"), pursuant to which the Merger Sub merged with and into X4, with X4 surviving the merger as a wholly-owned subsidiary of Arsanis. The transactions described in the foregoing sentence may be referred to in these consolidated financial statements as "the Merger."

The transaction was accounted for as a reverse merger in accordance with accounting principles generally accepted in the United States of America ("GAAP"). Under this method of accounting, X4 was deemed to be the accounting acquirer for financial reporting purposes. This determination was primarily based on the facts that, immediately following the Merger: (i) the Company's stockholders own a substantial majority of the voting rights in the combined organization, (ii) the Company designated a majority of the members of the initial board of directors of the combined organization and (iii) the Company's senior management hold all key positions in the senior management of the combined organization. Accordingly, for accounting purposes, the business combination was treated as the equivalent of X4 issuing stock to acquire the net assets of Arsanis. As a result, as of the closing date of the Merger, the net assets of Arsanis were recorded at their acquisition-date fair values in the financial statements of the Company and the reported operating results prior to the business combination are those of the Company. In addition, transaction costs incurred by the Company in connection with the business combination have been expensed as incurred.

On March 13, 2019, Arsanis, X4 and Merger Sub completed the Merger pursuant to the terms of the Merger Agreement. Pursuant to the terms of the Merger Agreement, each outstanding share of X4's common stock and preferred stock was exchanged for 0.5702 shares of Arsanis's common stock (the "Exchange Ratio"). In addition, all outstanding options exercisable for common stock and warrants exercisable for convertible preferred stock of X4 became options and warrants exercisable for the same number of shares of common stock of Arsanis multiplied by the Exchange Ratio. In connection with the Merger, X4 changed its name to X4 Therapeutics, Inc. Following the closing of the Merger, X4 Therapeutics, Inc. became a wholly-owned subsidiary of the Company, which changed its name to X4 Pharmaceuticals, Inc. As used herein, the words "the Company" refers to, for periods following the Merger, X4 Pharmaceuticals, Inc. (formerly Arsanis, Inc.), together with is direct and indirect subsidiaries, and for periods prior to the Merger, X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals, Inc.), and its direct and indirect subsidiaries, as applicable.

Immediately following the Merger, stockholders of X4 owned approximately 64% of the combined organization's outstanding common stock. On March 14, 2019, the combined organization's common stock began trading on The Nasdaq Capital Market under the ticker symbol "XFOR."

Principles of Consolidation— The consolidated financial statements include the accounts of the Company and its wholly-owned subsidiaries, including X4 Pharmaceuticals (Austria) GmbH, which is incorporated in Vienna, Austria and was formerly named Arsanis Biosciences GmbH ("X4 GmbH"), and X4 Therapeutics, Inc. All significant intercompany accounts and transactions have been eliminated.

Reverse Stock Split— On March 13, 2019, immediately following the closing of the Merger, the Company effected a 1-for-6 reverse stock split of its common stock (the "Reverse Stock Split"). Accordingly, all share and per share amounts for all periods presented in the accompanying consolidated financial statements and notes thereto have been adjusted retroactively, where applicable, to reflect the Reverse Stock Split. No fractional shares were issued in connection with the Reverse Stock Split. Unless otherwise noted, all references to common stock share and per share amounts have also been adjusted to reflect the exchange ratio of 0.5702.

Going Concern Assessment— In accordance with Accounting Standards Update ("ASU") No. 2014-15, *Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern (Subtopic 205-40)*, the Company has evaluated whether there are certain conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date that the consolidated financial statements are issued. As of March 12, 2020, the issuance date of these consolidated financial statements, the Company expects that its cash and cash equivalents will be sufficient to fund its forecasted operating expenses, capital expenditure requirements and debt service payments for at least the next twelve months from the issuance date of these financial statements.

Since its inception, the Company has incurred significant operating losses and negative cash flows from operations. The Company has not yet commercialized any products and does not expect to generate revenue from the commercial sale of any products for several years, if at all. The Company expects that its research and development and general and administrative expenses will continue to increase and, as a result, will need additional capital to fund its future operations, which it may raise through a combination of equity offerings, debt financings, other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements.

The Company has funded its operations to date primarily with proceeds from sales of common stock, warrants and prefunded warrants for the purchase of shares of preferred stock and shares of common stock, sales of preferred stock, proceeds from the issuance of convertible debt and borrowings under loan and security agreements. In 2019, the Company completed a merger with Arsanis and acquired its $26.4 million of cash, cash equivalents and restricted cash. In addition, in 2019, the Company raised $139.4 million, net of offering costs, through public offerings of common stock, prefunded warrants to purchase shares of common stock, and accompanying warrants to purchase shares of common stock. In June 2019, the Company received $9.8 million in net proceeds as a result of refinancing its loan agreement (the "Hercules Loan Agreement") with Hercules Capital, Inc. ("Hercules"), under which, subject to approval by Hercules, an additional $5.0 million is available for borrowing through December 15, 2020 and an additional term loan of $10.0 million is available through June 15, 2022. Principal payments under the Hercules Loan Agreement commence in February 2022.

If the Company is unable to obtain future funding when needed, the Company may be forced to delay, reduce or eliminate some or all of its research and development programs, product portfolio expansion or pre-commercialization efforts, which could adversely affect its business prospects, or the Company may be unable to continue operations. There is no assurance that the Company will be successful in obtaining sufficient funding on terms acceptable to the Company to fund continuing operations, if at all.

2. Summary of Significant Accounting Policies

Use of Estimates— The preparation of the Company's consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the consolidated financial statements, and the reported amounts of expenses during the reporting period. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to, the accrual of research and development expenses, the valuation of intangible assets acquired in business combinations, the valuations of common stock prior to the Merger, the valuation of stock options, preferred stock warrants (and the resulting preferred stock warrant liability), derivative instruments (and the resulting derivative liabilities), valuation of lease liabilities and the constraint of variable consideration from revenue transactions. The Company bases its estimates on historical experience, known trends and other market-specific or other relevant factors that it believes to be reasonable under the circumstances. On an ongoing basis, management evaluates its estimates when there are changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results could differ from those estimates.

Foreign Currency and Currency Translation— For the Company's subsidiaries that transact in a functional currency other than the U.S. dollar, assets and liabilities are translated at current exchange rates as of the balance sheet date while income and expenses are translated at the average exchanges rates for the period. Adjustments resulting from the translation of the financial statements of the Company's foreign operations into U.S. dollars are excluded from the determination of net loss and are recorded in accumulated other comprehensive loss, a component of stockholders' equity (deficit).

Concentrations of Credit Risk and Significant Suppliers— Financial instruments that potentially subject the Company to concentrations of credit risk consist principally of cash and cash equivalents and research and development incentive receivables. The Company generally maintains cash balances in various operating accounts at financial institutions that management believes to be of high credit quality in amounts that may exceed federally insured limits. The Company has not experienced losses related to its cash and cash equivalents.

The Company is dependent on third-party manufacturers to supply products for research and development activities in its programs. The Company relies and expects to continue to rely on a small number of manufacturers to supply it with its requirements for the active pharmaceutical ingredients and formulated drugs related to these programs. These programs could be adversely affected by a significant interruption in these manufacturing services or in the supply of active pharmaceutical ingredients and formulated drugs.

Cash and Cash Equivalents— The Company considers all highly liquid investments with maturities of three months or less at the date of purchase to be cash equivalents. Cash equivalents consisted of money market funds as of December 31, 2019 and 2018.

Restricted Cash—

(in thousands)	As of December 31, 2019	As of December 31, 2018
Letter of credit security: Cambridge lease	$ 264	$ 264
Letter of credit security: Waltham lease	250	—
Letter of credit security: Vienna Austria lease	94	—
Letter of credit security: Allston lease	1,144	—
Corporate credit card collateral	150	100
Total restricted cash (non-current)	$ 1,902	$ 364

In connection with the Company’s lease agreement for its facilities in Massachusetts and Austria, the Company maintains letters of credit, which are secured by restricted cash, for the benefit of the landlord. In addition, as of December 31, 2019, and 2018, the Company was required to maintain a separate cash balance of $150 thousand and $100 thousand, respectively, to collateralize corporate credit cards with a bank.

In accordance with the Company’s Amended and Restated Loan Agreement with Hercules, as further described in Note 8, the Company is required to maintain cash in an account accessible by the lender in an amount not less than 125% of the outstanding loan balance, or if the Company’s consolidated cash is lower than this amount, all of the Company’s cash other than $2.5 million. As of December 31, 2019, the Company maintained $25.0 million in an account accessible by the lender in accordance with the terms of Amended and Restated Loan Agreement.

The following table provides a reconciliation of cash, cash equivalents, and restricted cash reported within the consolidated balance sheets to the sum to the total of amounts shown in the Company’s consolidated statement of cash flows as of December 31, 2019, 2018 and 2017:

(in thousands)	December 31, 2019	December 31, 2018	December 31, 2017	December 31, 2016
Cash and cash equivalents	$ 126,184	$ 8,134	$ 26,684	$ 15,160
Restricted cash, non-current (included within other assets)	1,902	364	364	155
Total cash, cash equivalents and restricted cash	$ 128,086	$ 8,498	$ 27,048	$ 15,315

***Property and Equipment*—** Property and equipment are stated at cost less accumulated depreciation and amortization. Depreciation and amortization expense is recognized using the straight-line method over the estimated useful life of each asset, as follows:

	Estimated Useful Life
Office furniture	3 years
Computer equipment	3 years
Laboratory equipment	3 to 10 years
Leasehold improvements	Shorter of lease term or 10 years

Estimated useful lives are periodically assessed to determine if changes are appropriate. Maintenance and repairs are charged to expense as incurred. When assets are retired or otherwise disposed of, the cost of these assets and related accumulated depreciation or amortization are eliminated from the consolidated balance sheet and any resulting gains or losses are included in the consolidated statements of operations and comprehensive loss in the period of disposal. Costs for capital assets not yet placed into service are capitalized as construction-in-progress and depreciated once placed into service.

***Right-of-Use Assets and Leases*—** Effective January 1, 2019, the Company adopted Accounting Standards Codification ("ASC"), Topic 842, *Leases* ("ASC 842"), using the modified retrospective approach through a cumulative-effect adjustment and utilizing the effective date as its date of initial application, with prior periods unchanged and presented in accordance with the guidance in Topic 840, *Leases* ("ASC 840").

At the inception of an arrangement, the Company determines whether the arrangement contains a lease based on the unique facts and circumstances present. Leases with a non-cancellable term greater than one year are recognized on the balance sheet as right-of-use assets with associated current and non-current lease liabilities. The Company has elected not to recognize on the balance sheet leases with terms of one year or less. Options to renew a lease are not included in the Company's initial lease term assessment unless there is reasonable certainty that the Company will renew the lease. If a lease is cancellable without penalty, the Company excludes from the lease term periods following the cancellation notice period unless it is reasonably certain that the Company will not cancel the lease.

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. Certain adjustments to the right-of-use operating asset may be required for items such as incentives received or accrued rent. The interest rate implicit in lease contracts is typically not readily determinable. As a result, the Company utilizes its incremental borrowing rates, which are the rates it incurs to borrow on a collateralized basis over a similar term an amount equal to the lease payments in a similar economic environment. The Company has referenced the effective rate of its Hercules borrowings, as adjusted for differences terms, to determine calculate its incremental borrowing rate for each of its operating leases.

In accordance with the guidance in ASC 842, components of a lease are split into lease components and non-lease components. A policy election is available pursuant to which an entity may elect to not separate lease and non-lease components. Rather, each lease component and the related non-lease components are accounted for together as a single component. For new and amended leases beginning in 2019 and after, the Company has elected to account for the lease and non-lease components as a combined lease component for its office and laboratory building leases.

***Impairment of Long-Lived Assets*—** Long-lived assets consist of property and equipment. Long-lived assets to be held and used are tested for recoverability whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable. Factors that the Company considers in deciding when to perform an impairment review include significant underperformance of the business in relation to expectations, significant negative industry or economic trends and significant changes or planned changes in the use of the assets. If an impairment review is performed to evaluate a long-lived asset group for recoverability, the Company compares forecasts of undiscounted cash flows expected to result from the use and eventual disposition of the long-lived asset group to its carrying value. An impairment loss would be recognized in loss from operations when estimated undiscounted future cash flows expected to result from the use of an asset group are less than its carrying amount. The impairment loss would be based on the excess of the carrying value of the impaired asset group over its fair value. To date, the Company has not recorded any material impairment losses on long-lived assets.

Goodwill— Business combinations are accounted for under the acquisition method. The total purchase price of an acquisition is allocated to the underlying identifiable net assets, based on their respective estimated fair values as of the acquisition date. Determining the fair value of assets acquired and liabilities assumed requires management's judgment and often involves the use of significant estimates and assumptions, including assumptions with respect to future cash inflows and outflows, probabilities of success, discount rates, and asset lives, among other items. Assets acquired and liabilities assumed are recorded at their estimated fair values. The excess of the purchase price over the estimated fair values of the net assets acquired is recorded as goodwill.

Goodwill is tested quantitatively for impairment at the reporting unit level annually in the fourth quarter, or more frequently when events or changes in circumstances indicate that the asset might be impaired. Examples of such events or circumstances include, but are not limited to, a significant adverse change in legal or business climate, an adverse regulatory action or unanticipated competition.

The Company has determined that it operates in a single operating segment and has a single reporting unit. To perform its quantitative test, the Company compares the fair value of the reporting unit to its carrying value. If the fair value of the reporting unit exceeds the carrying value of its net assets, goodwill is not impaired, and no further testing is required. If the fair value of the reporting unit is less than the carrying value, the Company measures the amount of impairment loss, if any, as the excess of the carrying value over the fair value of the reporting unit. The Company determined that goodwill was not impaired as of December 31, 2019 base on its quantitative test.

Intangible Assets— In connection with the Merger, the Company acquired certain in-process research and development ("IPR&D") assets, which were classified as indefinite-lived intangible assets. Acquired IPR&D represents the fair value assigned to research and development assets that the Company acquires and have not been completed at the acquisition date. The fair value of IPR&D acquired in a business combination is recorded on the Company's consolidated balance sheets at the acquisition-date fair value and is determined by estimating the costs to develop the technology into commercially viable products, estimating the resulting revenue from the projects, and discounting the projected net cash flows to present value. IPR&D is not amortized, but rather is reviewed for impairment on an annual basis or more frequently if indicators of impairment are present, until the project is completed, abandoned or transferred to a third party.

The projected discounted cash flow models used to estimate the Company's IPR&D reflect significant assumptions regarding the estimates a market participant would make in order to evaluate a drug development asset, including the following:

- Probability of successfully completing clinical trials and obtaining regulatory approval;
- Market size, market growth projections, and market share;
- Estimates of future cash flows from potential milestone payments and royalties related to out-licensed product sales; and
- A discount rate reflecting the Company's weighted average cost of capital and specific risk inherent in the underlying assets.

During the year ended December 31, 2019, the Company entered into an out-licensing arrangement with a third party that transferred the rights to develop and commercialize one of the programs underlying an IPR&D intangible asset. In addition, the Company entered into amended out-licensing option agreements with a third party who had previously entered into an option agreement with Arsanis to license the rights to develop and commercialize two other programs underlying the IPR&D intangible assets. Following the amendment to these option agreements, the options were exercised by the third party and the in-process research and development programs were out-licensed to the third party. As of December 31, 2019, all programs underlying IPR&D intangible assets acquired in the Merger were transferred to these third parties and the Company has no continuing involvement in any ongoing research and development activities associated with the programs. As a result of the transfer of the IPR&D projects to third parties, the Company derecognized the IPR&D intangibles asset through a charge to "loss on transfer of nonfinancial assets" during 2019. (See Note 16)

Deferred Rent— The Company's lease agreements include payment escalations and lease incentives (including a leasehold improvement tenant allowance). For periods prior to January 1, 2019, these payments were accrued or deferred as appropriate such that rent expense was recognized on a straight-line basis over the respective lease terms. Effective January 1, 2019, upon the adoption of ASC 842, deferred rent was reclassified as a reduction to the applicable right-of-use asset as further described in Note 9.

Fair Value Measurements— Certain assets and liabilities of the Company are carried at fair value under GAAP. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

- Level 1—Quoted prices in active markets for identical assets or liabilities.
- Level 2—Observable inputs (other than Level 1 quoted prices), such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities, or other inputs that are observable or can be corroborated by observable market data.
- Level 3—Unobservable inputs that are supported by little or no market activity that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

Prior to the Merger, the Company's preferred stock warrant liability, derivative liability and preferred stock repurchase liability were carried at fair value, determined according to Level 3 inputs in the fair value hierarchy described above (see Note 5). The Company's cash equivalents, consisting of money market funds invested in U.S. Treasury securities, are carried at fair value, determined based on Level 1 and Level 2 inputs in the fair value hierarchy described above. The carrying values of the Company's accounts payable and accrued expenses approximate their fair values due to the short-term nature of these liabilities. The carrying value of the Company's outstanding loan and security agreement with Hercules approximates its fair value at December 31, 2019 because the debt bears interest at a variable market rate and the Company's credit risk has not materially changed since the inception of the agreement.

Segment Information— The Company manages its operations as a single operating segment for the purposes of assessing performance and making operating decisions. The Company's focus is on the research, development and commercialization of novel therapeutics for the treatment of rare diseases.

Revenue Recognition— Effective January 1, 2018, the Company adopted ASC Topic 606, *Revenue from Contracts with Customers* ("ASC 606"), as amended, using the modified retrospective transition method. The modified retrospective method requires that the cumulative effect of initially applying ASC 606 be recognized as an adjustment to the opening balance of retained earnings or accumulated deficit of the annual period that includes the date of initial application. The Company had no arrangements that were in the scope of ASC 606 on January 1, 2018 and thus there was no impact to the consolidated financial statements as a result of the adoption. This standard applies to all contracts with customers, except for contracts that are within the scope of other standards, such as collaboration arrangements and leases.

The Company's revenues are generated primarily through research, development and commercialization agreements. The terms of these agreements may contain multiple promised goods and services, which may include (i) licenses, or options to obtain licenses, to the Company's technology, and (ii) in certain cases, services in connection with the manufacturing of preclinical and clinical materials. Payments to the Company under these arrangements typically include one or more of the following: non-refundable, upfront license fees; option exercise fees; milestone payments; payments for clinical and commercial product supply, and royalties on future product sales. To date, all revenue from these agreements has been fully constrained and, therefore, no revenue has been recognized on the consolidated statements of operations.

The Company analyzes its arrangements to assess whether such arrangements involve joint operating activities performed by parties that are both active participants in the activities and exposed to significant risks and rewards dependent on the commercial success of such activities and are therefore within the scope of ASC Topic 808, *Collaborative Arrangements* ("ASC 808"). This assessment is performed throughout the life of the arrangement based on changes in the responsibilities of all parties in the arrangement. For collaboration arrangements that are deemed to be within the scope of ASC 808, the Company first determines which elements of the collaboration are deemed to be within the scope of ASC 808 and those that are more reflective of a vendor-customer relationship and, therefore, within the scope of ASC 606. The Company's policy is generally to recognize amounts received from collaborators in connection with joint operating activities that are within the scope of ASC 808 as a reduction in research and development expense. To date, there have been no transactions within the scope of ASC 808.

Under ASC 606, the Company recognizes revenue when its customers obtain control of promised goods or services, in an amount that reflects the consideration which the Company determines it expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements that the Company determines are within the scope of ASC 606, the Company performs the following five steps: (1) identify the contract(s) with a customer; (2) identify the performance obligation(s) in the contract; (3) determine the transaction price; (4) allocate the transaction price to the performance obligation(s) in the contract; and (5) recognize revenue when (or as) the Company satisfies its performance obligation(s).

As part of the accounting for these arrangements, the Company must make significant judgments, including identifying performance obligations in the contract, estimating the amount of variable consideration to include in the transaction price and; allocating the transaction price to each performance obligation.

Once a contract is determined to be within the scope of ASC 606, the Company assesses the goods or services promised within the contract and determines those that are performance obligations. Arrangements that include rights to additional goods or services that are exercisable at a customer's discretion are generally considered options. The Company assesses if these options provide a material right to the customer and if so, they are considered performance obligations.

The Company assesses whether each promised good or service is distinct for the purpose of identifying the performance obligations in the contract. This assessment involves subjective determinations and requires management to make judgments about the individual promised goods or services and whether such are separable from the other aspects of the contractual relationship. Promised goods and services are considered distinct provided that:

i. the customer can benefit from the good or service either on its own or together with other resources that are readily available to the customer (that is, the good or service is capable of being distinct) and
ii. the entity's promise to transfer the good or service to the customer is separately identifiable from other promises in the contract (that is, the promise to transfer the good or service is distinct within the context of the contract).

In assessing whether a promised good or service is distinct, the Company considers factors such as the research, manufacturing and commercialization capabilities of the customer and the availability of the associated expertise in the general marketplace. The Company also considers the intended benefit of the contract in assessing whether a promised good or service is separately identifiable from other promises in the contract. If a promised good or service is not distinct, an entity is required to combine that good or service with other promised goods or services until it identifies a bundle of goods or services that is distinct.

The transaction price is then determined and allocated to the identified performance obligations in proportion to their standalone selling prices ("SSP") on a relative SSP basis. SSP is determined at contract inception and is not updated to reflect changes between contract inception and when the performance obligations are satisfied. Determining the SSP for performance obligations requires significant judgment. In developing the SSP for a performance obligation, the Company considers applicable market conditions and relevant entity-specific factors, including factors that were contemplated in negotiating the agreement with the customer and estimated costs. The Company validates the SSP for performance obligations by evaluating whether changes in the key assumptions used to determine the SSP will have a significant effect on the allocation of arrangement consideration between multiple performance obligations.

If the consideration promised in a contract includes a variable amount, the Company estimates the amount of consideration to which it will be entitled in exchange for transferring the promised goods or services to a customer. The Company determines the amount of variable consideration by using either the expected value method or the most-likely-amount method. The Company includes the unconstrained amount of estimated variable consideration in the transaction price. The amount included in the transaction price reflects the amount for which it is probable that a significant reversal of cumulative revenue recognized will not occur. At the end of each subsequent reporting period, the Company re-evaluates the estimated variable consideration included in the transaction price and any related constraint, and if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis in the period of adjustment.

In determining the transaction price, the Company adjusts consideration for the effects of the time value of money if the timing of payments provides the Company with a significant benefit of financing. The Company does not assess whether a contract has a significant financing component if the expectation at contract inception is such that the period between payment by the licensees and the transfer of the promised goods or services to the licensees will be one year or less. The Company assesses each of its revenue generating arrangements in order to determine whether a significant financing component exists and concluded that a significant financing component does not exist in any of its arrangements.

The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) each performance obligation is satisfied at a point in time or over time, and if over time based on the use of an output or input method.

At the inception of each arrangement that includes non-refundable payments for contingent milestones, including preclinical research and development, clinical development and regulatory, 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. At the end of each reporting period, the Company re-evaluates the probability of the achievement of contingent milestones and the likelihood of a significant reversal of such milestone revenue, 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 licensing revenue in the period of adjustment. This quarterly assessment may result in the recognition of revenue related to a contingent milestone payment before the milestone event has been achieved.

Research and Development Programs— Proceeds under the research and development incentive program from the Austrian government are recognized as other income in an amount equal to the qualifying expenses incurred in each period multiplied by the applicable reimbursement percentage. Incentive income recognized upon incurring qualifying expenses in advance of receipt of proceeds from research and development incentives is recorded in the consolidated balance sheet as research and development incentive receivable.

Research and Development Costs— Costs associated with internal research and development and external research and development services, including drug development and preclinical studies, are expensed as incurred. Research and development expenses include costs for salaries, employee benefits, subcontractors, facility-related expenses, depreciation and amortization, stock-based compensation, third-party license fees, laboratory supplies, and external costs of outside vendors engaged to conduct discovery, preclinical and clinical development activities and clinical trials as well as to manufacture clinical trial materials, and other costs. The Company recognizes external research and development costs based on an evaluation of the progress to completion of specific tasks using information provided to the Company by its service providers.

Nonrefundable advance payments for services to be received in the future for use in research and development activities are recorded as prepaid expenses. Such prepaid expenses are recognized as an expense when the related services have been performed, or when it is no longer expected that the goods will be delivered or the services rendered.

Patent Costs— All patent-related costs incurred in connection with filing and prosecuting patent applications are expensed as incurred due to the uncertainty about the recovery of the expenditure. Amounts incurred are classified as general and administrative expenses.

Debt Issuance Costs— Debt issuance costs consist of payments made to secure commitments under certain debt financing arrangements. These amounts are recognized as interest expense over the period of the financing arrangement using the effective interest method. If the financing arrangement is canceled or forfeited, or if the utility of the arrangement to the Company is otherwise compromised, these costs are recognized as interest expense immediately. The Company's consolidated financial statements present debt issuance costs related to a recognized debt liability as a direct reduction from the carrying amount of that debt liability.

Stock-Based Compensation— The Company measures all stock-based awards granted to employees and directors based on the fair value on the date of the grant and recognizes compensation expense for those awards, net of estimated forfeitures, over the requisite service period, which is generally the vesting period of the respective award. The Company issues stock-based awards with service-based vesting conditions and records the expense for these awards using the straight-line method. The Company has not issued any stock-based awards with performance-based vesting conditions.

Effective January 1, 2019, the Company adopted ASU No. 2018-7, *Compensation—Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting* ("ASU 2018-7"), which expands the scope of Topic 718 to include share-based payment awards to nonemployees. As a result, stock-based awards granted to non-employees are accounted for in the same manner as awards granted to employees and directors as described above. The impact of adopting this new guidance did not have a material impact on the Company's consolidated financial statements. Prior to the adoption of ASU 2018-7, for stock-based awards granted to non-employee consultants, compensation expense was recognized over the period

during which services were rendered by such nonemployee consultants until completed. At the end of each financial reporting period prior to completion of the service, the fair value of these awards was remeasured using the then-current fair value of the Company's common stock and updated assumption inputs in the Black-Scholes option-pricing model.

The Company classifies stock-based compensation expense in its consolidated statement of operations and comprehensive loss in the same manner in which the award recipient's payroll costs are classified or in which the award recipient's service payments are classified.

The Company recognizes compensation expense for only the portion of awards that are expected to vest. In developing a forfeiture rate estimate, the Company has considered its historical experience to estimate pre-vesting forfeitures for service-based awards. The impact of a forfeiture rate adjustment is recognized in full in the period of adjustment, and if the actual forfeiture rate is materially different from the Company's estimate, the Company may be required to record adjustments to stock-based compensation expense in future periods.

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model. Prior to March 13, 2019, the Company had been a private company and lacked company-specific historical and implied volatility information for its common stock. Therefore, the Company estimates its expected common stock price volatility based on the historical volatility of publicly traded peer companies and expects to continue to do so until it has adequate historical data regarding the volatility of its own traded stock price. The expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The expected term of stock options granted to non-employee consultants is equal to the contractual term of the option award. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield considers the fact that the Company has never paid cash dividends on common stock and does not expect to pay any cash dividends in the foreseeable future.

Preferred Stock Warrant Liability— Prior to the Merger with Arsanis, the Company classified warrants for the purchase of shares of its convertible preferred stock (see Note 11) as a liability on its consolidated balance sheets as these warrants are freestanding financial instruments that may have required the Company to transfer assets upon exercise. The warrant liability, which consisted of warrants for the purchase of Series A and Series B convertible preferred stock, was initially recorded at fair value upon the date of issuance of each warrant and was subsequently remeasured to fair value at each reporting date. Changes in the fair value of the preferred stock warrant liability are recognized as a component of other income (expense), net in the consolidated statement of operations and comprehensive loss. Concurrent with the closing of the Merger, all X4 preferred stock was converted to common stock and the X4 preferred stock warrants converted to warrants for the purchase of Arsanis common stock. The Company assessed the features of the warrants and determined that they qualify for classification as permanent equity. Accordingly, the Company remeasured the warrants to fair value upon the closing of the Merger and reclassified the resulting warrant liability to additional paid-in capital.

Derivative Liabilities: Genzyme Contingent Payment— The Company's license agreement with Genzyme Corporation ("Genzyme") (see Note 4) contains a contingent payment obligation that required the Company to make a cash payment to Genzyme upon a change of control event of the Company. The contingent payment obligation met the definition of a derivative instrument as the contingent payment obligation was not clearly and closely related to its host instrument and was a cash-settled liability. Accordingly, the Company classified this derivative as a liability within other liabilities (non-current) on its consolidated balance sheet. The derivative liability was initially recorded at fair value on the date of entering into the license agreement and was subsequently remeasured to fair value at each reporting date. Changes in the fair value of this derivative liability were recognized as a component of other income (expense), net in the consolidated statement of operations and comprehensive loss. The Merger with Arsanis (see Note 1) qualified as a change of control event, as defined in the license agreement, but resulted in no payment being due to Genzyme under the license agreement. As a result, on March 13, 2019, the closing date of the Merger with Arsanis, the derivative liability was remeasured to fair value, which was $0, and subsequent changes in fair value will no longer be recognized in the consolidated statements of operations because the contingent payment obligation to Genzyme expired at that time.

Derivative Liabilities: Hercules Loan Redemption Feature— The Company's Hercules loan (see Note 8) contains a redemption feature that, upon an event of default, provides Hercules the option to accelerate and demand repayment of the debt, including a prepayment premium. The redemption feature meets the definition of a derivative instrument as the repayment of the debt contains a substantial premium, resulting in the redemption feature not being clearly and closely related to its host instrument. Accordingly, the Company classifies this derivative as a liability within other liabilities (non-current) on its consolidated balance

sheets. The derivative liability was initially recorded at fair value on the date of the Hercules Loan Agreement and is subsequently remeasured to fair value at each reporting date. Changes in the fair value of this derivative liability, which is included in other liabilities, are recognized as a component of other income (expense), net in the consolidated statement of operations and comprehensive loss. Changes in the fair value of this derivative liability will continue to be recognized until all amounts outstanding under the Hercules Loan Agreement are repaid or until the Hercules Loan Agreement is terminated. During the year ended December 31, 2019, there were no changes to the fair value of this liability.

Comprehensive Loss— Comprehensive loss includes net loss as well as foreign currency translation adjustments. For the year ended December 31, 2019, comprehensive loss includes $119 thousand of foreign currency translation adjustments.

Income Taxes— The Company accounts for income taxes using the asset and liability method, which requires the recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been recognized in the consolidated financial statements or in the Company's tax returns. Deferred tax assets and liabilities are determined based on the difference between the financial statement and tax basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Changes in deferred tax assets and liabilities are recorded in the provision for income taxes. The Company assesses the likelihood that its deferred tax assets will be recovered from future taxable income and, to the extent it believes, based upon the weight of available evidence, that it is more likely than not that all or a portion of the deferred tax assets will not be realized, a valuation allowance is established through a charge to income tax expense. Potential for recovery of deferred tax assets is evaluated by estimating the future taxable profits expected and considering prudent and feasible tax planning strategies.

The Company accounts for uncertainty in income taxes recognized in the consolidated financial statements by applying a two-step process to determine the amount of tax benefit to be recognized. First, the tax position must be evaluated to determine the likelihood that it will be sustained upon external examination by the taxing authorities. If the tax position is deemed more-likely-than-not to be sustained, the tax position is then assessed to determine the amount of benefit to recognize in the consolidated financial statements. The amount of the benefit that may be recognized is the largest amount that has a greater than 50% likelihood of being realized upon ultimate settlement. The provision for income taxes includes the effects of any resulting tax reserves, or unrecognized tax benefits, that are considered appropriate as well as the related net interest and penalties.

Net Loss per Share— For periods prior to the Merger with Arsanis on March 13, 2019, the Company followed the two-class method when computing net loss per share as the Company had issued shares that met the definition of participating securities. The two-class method determines net loss per share for each class of common and participating securities according to dividends declared or accumulated and participation rights in undistributed earnings. The two-class method requires income available to common stockholders for the period to be allocated between common and participating securities based upon their respective rights to receive dividends as if all income for the period had been distributed.

Basic net loss per share attributable to common stockholders is computed by dividing the net loss attributable to common stockholders by the weighted average number of shares of common stock outstanding for the period. Basic shares outstanding includes the weighted average effect of the Company's outstanding prefunded warrants, the exercise of which requires little or no consideration for the delivery of shares of common stock. Diluted net loss attributable to common stockholders is computed by adjusting net loss attributable to common stockholders to reallocate undistributed earnings based on the potential impact of dilutive securities. Diluted net loss per share attributable to common stockholders is computed by dividing the diluted net loss attributable to common stockholders by the weighted average number of shares of common stock outstanding for the period, including potential dilutive shares of common stock. For purpose of this calculation, outstanding stock options, convertible preferred stock and warrants to purchase shares of convertible preferred stock or common stock are considered potential dilutive shares of common stock.

The Company's convertible preferred stock contractually entitled the holders of such shares to participate in dividends but did not contractually require the holders of such shares to participate in losses of the Company. Accordingly, in periods in which the Company reports a net loss attributable to common stockholders, such losses are not allocated to such participating securities. In periods in which the Company reports a net loss attributable to common stockholders, diluted net loss per share attributable to common stockholders is the same as basic net loss per share attributable to common stockholders, since dilutive shares of common stock are not assumed to have been issued if their effect is anti-dilutive. The Company reported a net loss attributable to common stockholders for the years ended December 31, 2019, 2018 and 2017.

Recently Adopted Accounting Pronouncements

In February 2016, the Financial Accounting Standards Board ("FASB") issued ASU No. 2016-2, *Leases (Topic 842)* (“ASU 2016-2” or “ASC 842”)), which sets out the principles for the recognition, measurement, presentation and disclosure of leases for both parties to a contract (i.e., lessees and lessors). The new standard requires lessees to apply a dual approach, classifying leases as either finance or operating leases based on the principle of whether or not the lease is effectively a financed purchase by the lessee. This classification will determine whether lease expense is recognized based on an effective interest method or on a straight-line basis over the term of the lease. A lessee is also required to record a right-of-use asset and a lease liability for all leases with a term of greater than 12 months regardless of their classification. Leases with a term of 12 months or less may be accounted for similar to prior guidance for operating leases. The Company adopted the new lease standard as of the required effective date of January 1, 2019 using a modified retrospective transition approach applied to leases existing as of, or entered into after, January 1, 2019. The adoption had no impact on accumulated deficit. The new lease standard provides a number of optional practical expedients in transition. The Company applied the package of practical expedients to leases that commenced prior to the effective date, whereby it will elect to not reassess the following: (i) whether any expired or existing contracts contain leases; (ii) the lease classification for any expired or existing leases; and (iii) initial direct costs for any existing leases. The Company also elected the short-term lease recognition exemption for all leases that qualify, where a right-of-use asset or lease liability will not be recognized for short-term leases. Upon the adoption of ASC 842, the Company recorded $2.5 million of operating lease liabilities and $2.0 million of operating lease right-of-use assets on its consolidated balance sheets. The adoption did not have a material impact on the Company’s consolidated statement of operations and comprehensive loss or statement of cash flows.

In January 2017, the FASB issued ASU No. 2017-4, *Intangibles-Goodwill and Other (Topic 350): Simplifying the Test for Goodwill Impairment.* The new standard simplifies the subsequent measurement of goodwill by eliminating the second step of the goodwill impairment test. This ASU will be applied prospectively and is effective for annual or interim goodwill impairment tests in fiscal years beginning after December 15, 2019 with early adoption permitted. The Company adopted this guidance on January 1, 2019 and applied it to its annual impairment test for the year ending December 31, 2019. The adoption of this guidance did not have an impact on the Company's consolidated financial statements.

In April 2017, the FASB issued ASU 2017-8, *Receivables – Nonrefundable Fees and Other Costs* (“Subtopic 310-20”). The new standard amends the amortization period for certain purchased callable debt securities held at a premium by shortening the amortization period for the premium to the earliest call date. Subtopic 310-20 calls for a modified retrospective application under which a cumulative-effect adjustment will be made to retained earnings as of the beginning of the first reporting period in which the guidance is adopted. The Company adopted this guidance, effective January 1, 2019, and the adoption of this guidance did not have an impact on the Company's consolidated financial statements and related disclosures.

In July 2017, the FASB issued ASU No. 2017-11, *Earnings Per Share (Topic 260), Distinguishing Liabilities from Equity (Topic 480), Derivatives and Hedging (Topic 815) (Part I) Accounting for Certain Financial Instruments with Down Round Features (Part II) Replacement of the Indefinite Deferral for Mandatorily Redeemable Financial Instruments of Certain Nonpublic Entities and Certain Mandatorily Redeemable Noncontrolling Interests with a Scope Exception* (“ASU 2017-11”). Part I applies to entities that issue financial instruments such as warrants, convertible debt or convertible preferred stock that contain down-round features. Part II replaces the indefinite deferral for certain mandatorily redeemable noncontrolling interests and mandatorily redeemable financial instruments of nonpublic entities contained within ASC Topic 480 with a scope exception and does not impact the accounting for these mandatorily redeemable instruments. For public entities, ASU 2017-11 is effective for annual reporting periods beginning after December 15, 2018, including interim periods within those fiscal years. The Company adopted ASU 2017-11 as of the required effective date of January 1, 2019. The provisions of the new guidance related to accounting for financial instruments with down round features was taken into account by the Company in its evaluation of whether the Class B warrants issued in the November 2019 sale of common stock required liability classification. See Note 12.

In June 2018, the FASB issued ASU 2018-7, which superseded Subtopic 505-50, Equity—*Equity-Based Payments to Non-Employees* and amends ASC 718 to include share-based payments issued to non-employees for goods or services. Consequently, the accounting for share-based payments to non-employees and employees will be substantially aligned. The Company adopted ASU 2018-7 on January 1, 2019 and the adoption of this guidance did not have an impact on the Company's consolidated financial statements and related disclosures.

Recently Issued Accounting Pronouncements

In November 2018, the FASB issued ASU 2018-18, *Clarifying the Interaction between Topic 808 and Topic 606* (“ASU 2018-18”). ASU 2018-18 clarifies the interaction between the accounting guidance for collaborative arrangements and revenue from contracts with customers. The amendments become effective January 1, 2020 and were adopted by the Company retrospectively as of January 1, 2018, the date the Company adopted the new revenue guidance under ASC 606. The adoption of this guidance did not have a material impact on the Company's consolidated financial statements and related disclosures.

In June 2016, the FASB issued ASU 2016-13, *Credit Losses (Topic 326)* ("ASU 2016-13"). ASU 2016-13 requires that financial assets measured at amortized cost, such as trade receivables, be presented net of expected credit losses, which may be estimated based on relevant information such as historical experience, current conditions, and future expectation for each pool of similar financial asset. The new guidance requires enhanced disclosures related to trade receivables and associated credit losses. The Company adopted this guidance on January 1, 2020. The adoption of ASU 2016-13 is not expected to have a material impact on the Company's consolidated financial statements and related disclosures.

In August 2018, the FASB issued ASU 2018-15, *Intangibles-Goodwill and Other-Internal-Use Software (Subtopic 350-40): Customer’s Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That Is a Service Contract*, (“ASU 2018-15”). The amendments in this update align 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 (and hosting arrangements that include an internal-use software license). The accounting for the service element of a hosting arrangement that is a service contract is not affected by the amendments in this update. The new standard will be effective beginning January 1, 2020 and was adopted by the Company on that date. The adoption of ASU 2018-15 is not expected to have a material impact on the Company's consolidated financial statements and related disclosures.

In August 2018, the FASB issued ASU No. 2018-13, *Fair Value Measurement (Topic 820): Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement* (“ASU 2018-13”), which removes, adds and modifies certain disclosure requirements for fair value measurements in Topic 820. The Company will no longer be required to disclose the amount of and reasons for transfers between Level 1 and Level 2 of the fair value hierarchy as well as the valuation processes of Level 3 fair value measurements. The Company will be required to provide additional disclosure related to the changes in unrealized gains and losses included in other comprehensive loss for recurring Level 3 fair value measurements and the range and weighted average of assumptions used to develop significant unobservable inputs for Level 3 fair value measurements. ASU 2018-13 is effective for the Company on January 1, 2020 and was adopted on that date. The adoption of ASU 2018-13 is not expected to have an impact on the Company's consolidated financial statements and related disclosures.

In December 2019, the FASB issued ASU 2019-12, *Simplifying the Accounting for Income Taxes* ("ASU 2019-12"). ASU 2019-12 simplifies the accounting for income taxes, including the methodology for calculating income taxes in an interim period and the recognition of deferred tax liabilities for outside basis differences. ASU 2019-12 is effective for fiscal years beginning after December 15, 2020, including interim periods within those fiscal years. The Company does not anticipate that the adoption of ASU 2019-12 will have a material impact on its consolidated financial statements and related disclosures.

3. Merger Accounting

On March 13, 2019, the Company completed its merger with Arsanis. Based on the Exchange Ratio of 0.5702 , immediately following the Merger, former Arsanis stockholders, Arsanis option holders and other persons holding securities or other rights directly or indirectly convertible, exercisable or exchangeable for Arsanis common stock owned approximately 32.9% of the outstanding capital stock of the combined organization on a fully diluted basis, and former X4 stockholders, holders of options or warrants to acquire X4 capital stock and other persons holding securities and other rights directly or indirectly convertible, exercisable or exchangeable for X4 capital stock owned approximately 67.1% of the outstanding capital stock of the combined organization on a fully diluted basis. At the closing of the Merger, all shares of X4 common stock and X4 preferred stock then outstanding were exchanged for Arsanis common stock.

In addition, pursuant to the terms of the Merger Agreement, the Company, for accounting purposes, assumed all outstanding stock options to purchase shares of Arsanis common stock at the closing of the Merger. At the closing of the Merger, such stock options became options to purchase an aggregate of 271,230 shares of the Company’s common stock after giving effect to the Reverse Stock Split.

The total purchase price paid in the Merger has been allocated to the tangible and intangible assets acquired and liabilities assumed of Arsanis based on their fair values as of the completion of the Merger, with the excess allocated to goodwill. The following summarizes the preliminary estimate of the purchase price paid in the Merger (in thousands, except share and per share amounts):

Number of shares of the combined organization owned by Arsanis stockholders [1]		2,440,582
Multiplied by the fair value per share of Arsanis common stock [2]	$	18.66
Fair value of consideration issued it effect the Merger	$	45,541
Fair value of replacement awards held by former employees, board of directors and consultants of Arsanis that were vested as of the Merger.		817
Purchase price:	$	46,358

[1] The number of shares of 2,440,582 represents the historical 14,643,737 shares of Arsanis common stock outstanding immediately prior to the closing of the Merger, adjusted for the Reverse Stock Split.

[2] Based on the last reported sale price of Arsanis common stock on the Nasdaq Global Market on March 13, 2019, the closing date of the Merger, and gives effect to the Reverse Stock Split.

The following summarizes the allocation of the purchase price to the net tangible and intangible assets acquired (in thousands):

Cash, cash equivalents and restricted cash	$	26,406
Other current assets		2,147
Property and equipment, net		68
IPR&D indefinite-lived intangible assets		4,900
Other assets, non-current		486
Current liabilities		(5,205)
Loans payable		(8,713)
Other liabilities, non-current		(840)
Goodwill		27,109
Purchase price	$	46,358

The goodwill of $27.1 million is not tax deductible and represents the excess of the consideration paid over the fair value of assets acquired and liabilities assumed. Goodwill is mainly attributable to the enhanced value of the combined company, as reflected in the increase in market value of the shares of Arsanis common stock following the announcement of the Merger with X4. During the quarter ended June 30, 2019, goodwill was adjusted by $298 thousand to reflect a change in the allocation of purchase price to the fair value of an acquired operating lease as of March 13, 2019. There have been no other changes in the value of goodwill from the acquisition date to December 31, 2019, and goodwill was not impaired as of December 31, 2019 based on management's annual quantitative impairment test.

The Company incurred costs directly related to the Merger of approximately $1 million, which were recorded in general and administrative expenses, for the twelve months ended December 31, 2019.

The in-process research and development intangible assets represent the acquisition-date fair value of three development programs acquired from Arsanis, which the Company refers to as the ASN200, ASN300 and ASN500 programs. The fair value of the IPR&D intangible assets was based on assumptions that market participants would use in pricing the assets, based on the most advantageous market for the assets assuming highest and best use. The fair value was determined by estimating the resulting revenue from out-license arrangements related to the projects, and discounting the projected net cash flows to present value using a weighted average discount rate of 20%. These IPR&D intangible assets are not amortized, but rather are reviewed for impairment on an annual basis or more frequently if indicators of impairment are present, until the project is completed, abandoned or transferred to a third party. As further described in Note 16, during the year ended December 31, 2019, the Company entered into arrangements with two third parties that transferred the rights to develop and commercialize the programs underlying the IPR&D intangible assets. Accordingly, the Company derecognized the IPR&D intangible assets through a charge to "loss on transfer of nonfinancial assets" during the year ended December 31, 2019.

The following supplemental pro forma information presents the Company's financial results as if the acquisition of Arsanis had occurred on January 1, 2018 (unaudited)

	Year Ended December 31,	
(in thousands)	2019	2018
Revenue	$ —	$ 3,500
Net loss	$ (57,820)	$ (76,248)

The above unaudited pro forma information was determined based on the historical GAAP results of the Company and Arsanis. The pro forma consolidated results are not necessarily indicative of what the Company's consolidated results of operations would have been if the acquisition was completed on January 1, 2018. The pro forma consolidated net loss includes pro forma adjustments primarily relating to the reclassification of transaction costs and severance payments directly related to the closing of the Merger of $2.7 million from the twelve months ended December 31, 2019 to the twelve months ended December 31, 2018.

4. License, Collaboration, and Funding Agreements

Genzyme Agreement

In July 2014, the Company entered into a license agreement (the "Genzyme Agreement") with Genzyme pursuant to which the Company was granted an exclusive license to certain patents and intellectual property owned or controlled by Genzyme related to the CXCR4 receptor to develop and commercialize products containing licensed compounds (including but not limited to mavorixafor) for all therapeutic, prophylactic and diagnostic uses, with the exception of autologous and allogenic human stem cell therapy. Under the terms of the Genzyme Agreement, the Company is obligated to use commercially reasonable efforts to develop and commercialize licensed products for use in the field in the United States and at least one other major market country. The Company has the right to grant sublicenses of the licensed rights that cover mavorixafor to third parties.

In exchange for these rights, in August 2014, the Company made an upfront payment of $50 thousand to Genzyme. The Company accounted for the acquisition of technology as an asset acquisition because it did not meet the definition of a business. The Company recorded the upfront payment as research and development expense in the consolidated statement of operations and comprehensive loss because the acquired technology represented in-process research and development and had no alternative future use. In August 2015, as a result of the closing of the Company's Series A preferred stock financing, the Company made an additional cash payment of $300 thousand to Genzyme and issued to Genzyme 107,371 shares of its common stock, as adjusted for the 1-for-6 Reverse Stock Split and Exchange Ratio, each as required by the Genzyme Agreement. The $300 thousand payment and the $734 thousand fair value of the 107,371 shares of common stock issued to Genzyme were recorded as research and development expense in the consolidated statements of operations and comprehensive loss for the year ended December 31, 2015. Prior to the Merger with Arsanis, Genzyme had the right to require the Company to repurchase all, but not less than all, of these shares of common stock at any time during the term of the Genzyme Agreement for a price of $0.01 per share. Due to this redemption feature, the shares of common stock issued to Genzyme were classified outside of stockholders' deficit on the consolidated balance sheets as of December 31, 2018. On March 13, 2019, the closing date of the Merger with Arsanis, these redeemable shares of common stock were exchanged for shares of common stock and, as a result, the fair value of the shares was reclassified to permanent equity.

Under the Genzyme Agreement, the Company is obligated to pay Genzyme milestone payments in the aggregate amount of up to $25.0 million, contingent upon the achievement by the Company of certain clinical-stage regulatory and sales milestones with respect to licensed products. The Company is also obligated to pay Genzyme tiered royalties based on net sales of licensed products that the Company commercializes under the agreement. The obligation to pay royalties for each licensed product expires on a country-by-country basis on the latest of (i) the expiration of licensed patent rights that cover that licensed product in that country, (ii) the expiration of regulatory exclusivity in that country and (iii) ten years after the first commercial sale of such licensed product in that country. Royalty rates are subject to reduction under the agreement in specified circumstances, including in any country if the Company is required to obtain a license from any third party to the extent the Company's patent rights might infringe the third party's patent rights, if a licensed product is not covered by a valid claim in that country or if sales of generic products reach certain thresholds in that country. If the Company enters into a sublicense under the Genzyme Agreement, the Company will be obligated to pay Genzyme a percentage of certain upfront fees, maintenance fees, milestone payments and royalty payments paid to the Company by the sublicensee.

Under the Genzyme Agreement, the Company will itself manufacture and supply, or enter into manufacturing or supply agreements with Genzyme or third parties to manufacture and supply, clinical and commercial supplies of licensed compounds and each licensed product. The Company is also responsible for all costs related to the filing, prosecution and maintenance of the licensed patent rights.

The Genzyme Agreement will remain in effect until the expiration of the royalty term in all countries for all licensed products. The Genzyme Agreement may be terminated by either party with at least 90 days' notice in the event of material breach by the other party that remains uncured for 90 days, by either party for insolvency or bankruptcy of the other party, immediately by Genzyme if the Company challenges the licensed patents, or immediately by the Company if a material safety issue arises.

During the twelve months ended December 31, 2019, 2018 and 2017, the Company did not incur any payment obligations to Genzyme under the Genzyme Agreement.

Georgetown Agreement

In December 2016, the Company entered into a license agreement (the "Georgetown Agreement") with Georgetown University ("Georgetown") pursuant to which the Company obtained an exclusive, worldwide license to make, have made, use, sell, offer for sale and import of products covered by patent rights co-owned by Georgetown. The rights licensed to the Company are for all therapeutic, prophylactic and diagnostic uses in all disease indications in humans and animals.

Under the terms of the Georgetown Agreement, the Company paid a one-time only, upfront fee of $50 thousand and the Company may be required to make milestone payments of up to an aggregate of $800 thousand related to commercial sales of a product. The Company recorded the upfront payment as research and development expense in the consolidated statement of operations and comprehensive loss because the acquired technology represented in-process research and development and had no alternative future use.

Under the Georgetown Agreement, the Company is solely responsible for all development and commercialization activities and costs in its respective territories. The Company is also responsible for all costs related to the filing, prosecution and maintenance of the licensed patent rights.

The term of the Georgetown Agreement will continue until the expiration of the last valid claim within the patent rights covering the product. Georgetown may terminate the agreement in the event (i) the Company fails to pay any amount and fails to cure such failure within 30 days after receipt of notice, (ii) the Company defaults in its obligation to obtain and maintain insurance and fails to remedy such breach within 45 days after receipt of notice, or (iii) the Company declares insolvency or bankruptcy. The Company may terminate the Georgetown Agreement at any time upon at least 60 days' written notice.

During the twelve months ended December 31, 2019, 2018 and 2017, the Company did not incur any payment obligations to Georgetown under the Georgetown Agreement and no milestone payments were made or due under the Georgetown Agreement.

Beth Israel Deaconess Medical Center Agreement

In December 2016, the Company entered into a license agreement (the "BIDMC Agreement") with Beth Israel Deaconess Medical Center ("BIDMC"), pursuant to which the Company obtained an exclusive, worldwide license to make, have made, use, sell, offer for sale and import products covered by patent rights co-owned by BIDMC. The rights licensed to the Company are for all fields of use.

Under the terms of the BIDMC Agreement, the Company paid a one-time, upfront fee of $20 thousand and the Company is responsible for all future patent prosecution costs. The Company recorded the upfront payment as research and development expense in the consolidated statement of operations because the acquired technology represented in-process research and development and had no alternative future use.

The term of the BIDMC Agreement will continue until the expiration of the last valid claim within the patent rights covering the licensed products. BIDMC may terminate the agreement in the event (i) the Company fails to pay any amount and fails to cure such failure within 15 days after receipt of notice, (ii) the Company is in material breach of any material provision of the BIDMC Agreement and fails to remedy such breach within 60 days after receipt of notice, or (iii) the Company declares insolvency or bankruptcy. The Company may terminate the BIDMC Agreement at any time upon at least 90 days' written notice.

The Company did not incur any payment obligations under the BIDMC Agreement during the twelve months ended December 31, 2019, 2018 and 2017.

Research and Development Incentive Program

The Company's wholly-owned subsidiary X4 GmbH, which was acquired in March 2019 in the Merger, participates in a research and development incentive program provided by the Austrian government whereby the Company is entitled to reimbursement by the Austrian government for a percentage of qualifying research and development expenses incurred by the Company's subsidiary in Austria. Under the program, the reimbursement rate for qualifying research and development expenses incurred by the Company through its subsidiary in Austria is 14% for the current year.

The Company recognizes incentive income from Austrian research and development incentives when qualifying expenses have been incurred, there is reasonable assurance that the payment will be received, and the consideration can be reliably measured. Management has assessed the Company's research and development activities and expenditures to determine which activities and expenditures are likely to be eligible under the research and development incentive program described above. At each reporting date, management estimates the reimbursable incentive income available to the Company based on available information at the time.

As of December 31, 2019, the amount due under the program was $2.0 million, which is included in research and development incentive receivable in the consolidated balance sheet and of which $1.5 million was collected in the first quarter of 2020. During the year ended December 31, 2019, the Company recorded $446 thousand of income related to the program on the consolidated statement of operations and comprehensive loss within "other income".

Abbisko Agreement

In July 2019, the Company entered into a license agreement (the "Abbisko Agreement") with Abbisko Therapeutics Co., Ltd. ("Abbisko"). Under the terms of the Abbisko Agreement, the Company granted Abbisko the exclusive right to develop, manufacture and commercialize mavorixafor in mainland China, Taiwan, Hong Kong and Macau (referred to in the Abbisko Agreement as the "Abbisko Territory"). The agreement provides Abbisko with the exclusive rights in the Abbisko Territory to develop and commercialize mavorixafor in combination with checkpoint inhibitors or other agents in multiple oncology indications. Pancreatic cancer, ovarian cancer and triple negative breast cancer will be explored initially. The Company retains the full rest-of-world rights to develop and commercialize mavorixafor outside of Greater China for all indications and the ability to utilize data generated pursuant to the Abbisko collaboration for rest-of-world development. In addition, Abbisko has the right of first refusal if the Company determines to pursue additional products in the Abbisko Territory. In accordance with the Abbisko Agreement, the Company has agreed to enter into separate agreements whereby the Company would provide Abbisko with a clinical supply and, if the product is commercialized in the Abbisko Territory, a commercial supply of the licensed compound.

Pursuant to the Abbisko Agreement, upon the closing of a qualified financing (as defined in the Abbisko Agreement), Abbisko will make a one-time, non-refundable, non-creditable financial milestone payment in the low single-digit millions to the Company. The Company is also eligible to receive potential development and regulatory milestone payments, which vary based on the number of indications developed, and potential commercial milestone payments based on annual net sales of mavorixafor-based licensed products. Assuming mavorixafor is developed by Abbisko in six indications, the Company would be entitled to milestone payments of up to $208.0 million, which will vary based on the ultimate sales, if any, of the approved licensed products. In addition, upon commercialization of mavorixafor in the Abbisko Territory, the Company is eligible to receive a tiered royalty, with a percentage range in the low double-digits, on net sales of approved licensed products. Abbisko is obligated to use commercially reasonable efforts to develop and commercialize mavorixafor in the Abbisko Territory. Abbisko has responsibility for all activities and costs associated with the further development, manufacture and commercialization of mavorixafor in the Abbisko Licensed Territory.

The Company evaluated the Abbisko Agreement under ASC 606 and determined the Abbisko Agreement contained a single performance obligation related to the exclusive license to develop and commercialize mavorixafor and the transfer of know-how that was satisfied at the inception of the arrangement. The transaction price related to the transfer of the license and know-how was fully constrained and the Company ascribed no transaction price to the development, regulatory and commercial milestones under the "most-likely amount" method. As part of its evaluation, the Company considered multiple factors: (i) the ability of Abbisko to achieve a qualified financing (as defined in the Abbisko Agreement) is dependent on many factors outside of its control (ii) regulatory approvals are outside of the control of Abbisko, and (iii) certain development and regulatory milestones are contingent upon the success of future clinical trials, if any, which is out of the control of Abbisko. Any consideration related to the

initial transfer of the license and know-how will be recognized when it is probable that Abbisko will achieve the related financial milestone. Any consideration related to development and regulatory milestones will be recognized when the corresponding milestones are achieved. Any consideration related to sales-based milestones (including royalties) will be recognized when the related sales occur as these amounts have been determined to relate predominantly to the license granted to Abbisko and therefore are recognized at the later of when the performance obligation is satisfied or the related sales occur.

The Company determined that the future sale of clinical and commercial supply are optional goods that will be subject to the customer's future purchasing decisions and do not represent performance obligations in the Abbisko Agreement. The Company concluded that the amount to be charged for the clinical supply will be reflective of market value and, therefore, the Abbisko Agreement does not provide a discount on such supply that would be accounted for as material right at the outset of the contract. In arriving at these conclusions, the Company considered the complexity of the manufacturing process for the licensed compound and the potential ability for Abbisko to obtain the compound directly from other manufactures in the future. The Company expects that it will recognize revenue at a point in time when such clinical supply (and commercial supply, if applicable) is delivered to Abbisko in the future.

The Company will re-evaluate the transaction price, including its estimated variable consideration for milestones included in the transaction price and all constrained amounts, in each reporting period and as uncertain events are resolved or other changes in circumstances occur.

5. Fair Value of Financial Assets and Liabilities

The following tables present information about the Company’s financial assets and liabilities measured at fair value on a recurring basis and indicate the level of the fair value hierarchy used to determine such fair values:

	Fair Value Measurements as of December 31, 2019 Using:			
(in thousands)	**Level 1**	**Level 2**	**Level 3**	**Total**
Assets:				
Cash equivalents—money market funds	$ 23,638	$ 39,999	$ —	$ 63,637
	$ 23,638	$ 39,999	$ —	$ 63,637

Liabilities: none

	Fair Value Measurements as of December 31, 2018 Using:			
(in thousands)	**Level 1**	**Level 2**	**Level 3**	**Total**
Assets:				
Cash equivalents—money market fund	$ —	$ 8,134	$ —	$ 8,134
	$ —	$ 8,134	$ —	$ 8,134
Liabilities:				
Preferred stock warrant liability	$ —	$ —	$ 4,947	$ 4,947
Derivative liability	—	—	201	201
	$ —	$ —	$ 5,148	$ 5,148

As of December 31, 2019 and December 31, 2018, there were no transfers between Level 1, Level 2 and Level 3.

The Company’s cash equivalents consisted of money market funds invested in U.S. Treasury securities. The money market funds were valued based on reported market pricing for the identical asset or by using inputs observable in active markets for similar securities, which represents a Level 2 measurement in the fair value hierarchy.

Valuation of Preferred Stock Warrant Liabilities— The preferred stock warrant liability in the table above consists of the fair values of (i) warrants to purchase shares of Series A convertible preferred stock that were issued in 2015 and shares of Series B convertible preferred stock that were issued in 2017 and 2018 in connection with the Company’s Series A and Series B convertible preferred stock financings, respectively (see Note 11), (ii) warrants to purchase shares of Series A convertible

preferred stock that were issued in 2016 in connection with the Company's entering into a loan and security agreement with Silicon Valley Bank (see Note 8) and (iii) warrants to purchase shares of Series B convertible preferred stock that were issued or were issuable in 2018 in connection with the Company's entering into the Hercules Loan Agreement (see Note 8). The liability associated with the warrants was recorded at fair value on the dates the warrants were issued and exercisable and was subsequently remeasured to fair value at each reporting date through December 31, 2018. Upon the closing of the Merger on March 13, 2019, all X4 preferred stock warrants were converted to warrants for Company's common stock and, as a result, the warrants were adjusted to fair value and reclassified to permanent equity. The aggregate fair value of the warrant liability was determined based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy.

The Company used various valuation methods, including the Monte Carlo method, the option-pricing method and the hybrid method (which is a combination of an option-pricing method and a probability-weighted expected return method), all of which incorporate assumptions and estimates, to value the preferred stock warrants. Estimates and assumptions impacting the fair value measurement include the fair value per share of the underlying shares of the Company's Series A and Series B convertible preferred stock, risk free interest rate, expected dividend yield, expected volatility of the price of the underlying preferred stock, and either the remaining contractual term of the warrants (except for warrants that would be automatically exercised upon an initial public offering, in which case the remaining estimated term to automatic exercise was used). The most significant assumption in the Monte Carlo method, the option-pricing method and the hybrid method impacting the fair value of the preferred stock warrants is the fair value of the Company's convertible preferred stock as of each remeasurement date. The Company determines the fair value per share of the underlying preferred stock by taking into consideration the most recent sales of its convertible preferred stock, results obtained from third-party valuations and additional factors that are deemed relevant. As of December 31, 2018, the fair value of the Series A convertible preferred stock was $1.70 per share and the fair value of the Series B convertible preferred stock was $1.86 per share. There were no warrants for the purchase of convertible preferred shares as of December 31, 2019 as all such warrants were converted to warrants for the purchase of common stock upon the Merger. The Company was a private company prior to the Merger and lacked company-specific historical and implied volatility information of its stock. Therefore, the Company had estimated its expected stock volatility based on the historical volatility of publicly traded peer companies for a term equal to the estimated remaining term of the warrants. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve for time periods approximately equal to the estimated remaining term of the warrants. The Company estimated a 0% expected dividend yield as the Company has never paid or declared dividends and does not intend to do so in the foreseeable future.

Valuation of Derivative Liability— The fair value of the derivative liability recognized in connection with the Genzyme Agreement (see Note 4) was determined based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy. The fair value of this derivative liability is reported within other liabilities on the consolidated balance sheets. The fair value of this derivative liability was estimated by the Company at each reporting date based, in part, on the results of third-party valuations, which were prepared using the option-pricing method or the hybrid method, each of which considered as inputs the type, timing and probability of occurrence of a change of control event, the potential amount of the payment under potential exit scenarios, the fair value per share of the underlying common stock and the risk-adjusted discount rate. As of December 31, 2018, the fair value of this derivative liability was $183 thousand. The Merger (see Note 1) qualified as a change of control event, as defined in the Genzyme Agreement, but results in no payment being due to Genzyme under the Genzyme Agreement. As a result, on March 13, 2019, the closing date of the Merger with Arsanis, this derivative liability was remeasured to fair value, which was zero, and subsequent changes in fair value will no longer be recognized in the consolidated statements of operations because the contingent payment obligation to Genzyme expired at that time.

The fair value of the derivative liability recognized in connection with the Hercules Loan Agreement (see Note 8) was determined based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy. The fair value of this derivative liability is reported within other liabilities on the consolidated balance sheets. The fair value of this derivative liability was estimated by the Company at each reporting date based, in part, on the results of third-party valuations, which were prepared based on a discounted cash flow model that considered the timing and probability of occurrence of a redemption upon an event of default, the potential amount of prepayment upon an event of default and the risk-adjusted discount rate. As of December 31, 2019 and December 31, 2018, the fair value of this derivative liability was immaterial.

The following table provides a roll-forward of the aggregate fair values of the Company's warrant liability and derivative liability, for which fair values are determined using Level 3 inputs:

(in thousands)	Preferred Stock Warrant Liability	Derivative Liability
Balance as of December 31, 2017	$ 1,245	$ 94
Issuance of warrants to purchase shares of Series B convertible preferred stock	304	—
Initial fair value of derivative liability in connection with the Hercules Loan Agreement	—	18
Change in fair value	3,398	89
Balance as of December 31, 2018	4,947	201
Change in fair value	288	(183)
Conversion of convertible preferred stock warrant into common stock warrant in connection with Merger	(5,235)	—
Balance at December 31, 2019	$ —	$ 18

6. Property and Equipment, Net

Property and equipment, net consisted of the following:

(in thousands)	December 31, 2019	December 31, 2018
Leasehold improvements	$ 299	$ 299
Furniture and fixtures	139	53
Computer equipment	37	56
Software	33	9
Lab equipment	159	—
	667	417
Less: Accumulated depreciation and amortization	(264)	(176)
	$ 403	$ 241

Depreciation and amortization expense related to property and equipment was approximately $103 thousand for each of the twelve months ended December 31, 2019 and 2018 and $71 thousand for the year ended December 31, 2017.

7. Accrued Expenses

Accrued expenses consisted of the following

(in thousands)	December 31, 2019	December 31, 2018
Accrued employee compensation and benefits	$ 2,916	$ 924
Accrued external research and development expenses	1,977	754
Accrued professional fees	1,347	1,324
Other	221	249
	$ 6,461	$ 3,251

8. Long-Term Debt

Long-term debt consisted of the following:

(in thousands)	December 31, 2019	December 31, 2018
Principal amount of long-term debt	$ 20,000	$ 10,000
Less: Current portion of long-term debt	—	(1,687)
Long-term debt, net of current portion	20,000	8,313
Debt discount, net of accretion	(317)	(226)
Cumulative accretion of final payment due at maturity	414	58
Long-term debt, including accretion, net of discount and current portion	$ 20,097	$ 8,145

SVB Loan Agreement

In October 2016, the Company entered into a loan and security agreement with Silicon Valley Bank ("SVB"), which the Company refers to as the SVB Loan Agreement, pursuant to which SVB made certain term loans available to the Company. The SVB Loan Agreement provided for a term loan of up to $6.0 million, which was borrowed by the Company in September 2017. Borrowings under the SVB Loan Agreement bore interest at a variable rate equal to 5.5% plus the greater of (i) 3.5% or (ii) *The Wall Street Journal* prime rate. In October 2018, in connection with entering into the Hercules Loan Agreement, the Company terminated the SVB Loan Agreement and repaid all amounts due under the SVB Loan Agreement of $4.7 million, including outstanding principal, prepayment premiums and accrued interest. The Company accounted for the termination of the SVB Loan Agreement as an extinguishment and recognized a loss of $229 thousand, which was calculated as the difference between the reacquisition price of the borrowings under the SVB Loan Agreement and the carrying value of the debt at the time of the extinguishment.

The Company recognized aggregate interest expense under the SVB Loan Agreement of $484 thousand and $490 thousand for the years ended December 31, 2018 and 2017, respectively, reflecting an effective interest rate of approximately 11.8% and 12.0%, respectively. As of October 19, 2018, the date of repayment of all borrowings under the SVB Loan Agreement, the contractual interest rate applicable to borrowings under the SVB Loan Agreement was 10.75%.

Hercules Loan Agreements

Hercules Loan Agreement and First Amendment

In October 2018, the Company entered into the Hercules Loan Agreement. The Hercules Loan Agreement provided for aggregate borrowings of up to $13.0 million, consisting of (i) a term loan of up to $8.0 million, which was available upon entering into the agreement, (ii) subject to specified financing conditions, an additional term loan of up to $2.0 million, available for borrowing from January 1, 2019 to March 31, 2019, and (iii) subject to specified financing conditions and the receipt of the second tranche term loan in the amount of $2.0 million described above, an additional term loan of up to $3.0 million, available for borrowing until March 31, 2019. In October 2018, the Company borrowed $8.0 million under the Hercules Loan Agreement. In December 2018, the Company entered into the First Amendment to the Hercules Loan Agreement (the "First Amendment"), which amended the available borrowing dates of the second tranche from between January 1, 2019 and March 31, 2019 to between December 11, 2018 and December 14, 2018 and amended the term loan maturity date to November 1, 2021. In December 2018, the Company borrowed the additional $2.0 million provided under the Hercules Loan Agreement, as amended by the First Amendment. In March 2019, the conditions necessary for borrowing the remaining $3.0 million under the Hercules Loan Agreement, as amended by the First Amendment, were not met and the borrowing capacity expired at that time.

In connection with entering into the Hercules Loan Agreement in October 2018, the Company issued to Hercules warrants for the purchase of 210,638 shares of Series B convertible preferred stock at an exercise price of $1.88 per share (which were subsequently converted to warrants for the purchase of 20,016 shares of common stock at an exercise price of $19.78 per share following the Merger). These warrants were immediately exercisable and expire in October 2028. In addition, in connection with entering into the First Amendment in December 2018, the Company agreed to issue to Hercules warrants for the purchase of a specified number of shares of convertible preferred stock at an aggregate exercise price of $99. On March 18, 2019, as a result of the closing of the Merger with Arsanis on March 13, 2019, the Company issued to Hercules warrants for the purchase of 5,000 shares of common stock of the combined organization at an exercise price of $19.80 per share, each of which reflected the share Exchange Ratio of 1-for-0.5702 applied in the Merger as well as the Reverse Stock Split effected by the combined organization on March 13, 2019. On October 19, 2018 and December 11, 2018, the dates the Company entered into the Hercules Loan Agreement and the First Amendment, respectively, the Company recorded the aggregate initial fair value of the warrants of $132 thousand as a preferred stock warrant liability, with a corresponding amount recorded as a debt discount on the Company's

consolidated balance sheet. As of March 13, 2019, and December 31, 2018, the fair value of the warrants were $326 thousand and $282 thousand, respectively. Upon the closing of the Merger, the warrants were converted to warrants for common stock and are no longer adjusted to fair value.

2019 Amended and Restated Loan Agreement

In June 2019, the Company refinanced the Hercules Loan Agreement, as amended, and entered into an Amended and Restated Loan and Security Agreement (the "2019 Loan Agreement") with Hercules. The 2019 Loan Agreement provides for aggregate maximum borrowings of $35.0 million, of which $10.0 million was previously outstanding. The Company agreed to borrow $20.0 million as of the closing date on June 27, 2019, including $10.0 million in new borrowings and $10.0 million rolled over from the previous agreement. An additional $5.0 million is available for borrowing through December 15, 2020 and, subject to approval by Hercules, and an additional term loan of $10.0 million is available through June 15, 2022. Borrowings under the 2019 Loan Agreement bear interest at a variable rate equal to the greater of (i) 8.75% or (ii) 8.75% plus *The Wall Street Journal* prime rate minus 6.0%. In an event of default, as defined in the 2019 Loan Agreement, and until such event is no longer continuing, the interest rate applicable to borrowings under the 2019 Loan Agreement would be increased by 4.0%.

Borrowings under the 2019 Loan Agreement are repayable in monthly interest-only payments through January 1, 2022, and in equal monthly payments of principal and accrued interest from February 1, 2022 until the maturity date of the loan, which is July 1, 2023. At the Company's option, the Company may prepay all, but not less than all, of the outstanding borrowings, subject to a prepayment premium of up to 2.0% of the principal amount outstanding as of the date of repayment. In addition, the 2019 Loan Agreement provides for payments by the Company to Hercules of (i) $0.8 million payable upon the earlier of November 1, 2021 or the repayment in full of all obligations under the 2019 Loan Agreement, and (ii) 4.0% of the aggregate principal drawn under the 2019 Loan Agreement payable upon the earlier of maturity or the repayment in full of all obligations under such agreement.

Borrowings under the 2019 Loan Agreement are collateralized by substantially all of the Company's personal property and other assets except for the Company's intellectual property (but including rights to payment and proceeds from the sale, licensing or disposition of the intellectual property). Under the 2019 Loan Agreement, the Company has agreed to affirmative and negative covenants to which it will remain subject until maturity or repayment of the loan in full. The covenants include (a) maintaining a minimum liquidity amount of the lesser of (i) 125% of the aggregate principal amount of outstanding borrowings under the June 2019 Loan Agreement and (ii) 100% of the Company and its consolidated subsidiaries' cash and cash equivalents (other than up to $2.5 million which may be held by an excluded subsidiary, as defined) in an account in which Hercules has a first priority security interest, as well as (b) restrictions on the Company's ability to incur additional indebtedness, pay dividends, encumber its intellectual property, or engage in certain fundamental business transactions, such as mergers or acquisitions of other businesses. The 2019 Loan Agreement also contains a covenant that requires the Company to repay its loan with Österreichische Forschungsförderungsgesellschaft mbH ("FFG") on or prior to December 31, 2019. The FFG Loan Agreement was settled during the three months ended September 30, 2019 as discussed further below.

In connection with entering into the Hercules Loan Agreement and the First Amendment, the Company had deferred $180 thousand associated with upfront (1) fees associated with entering into the agreement, (2) the fair value of the warrants issued and (3) the fair value of an embedded derivative associated with the accelerated redemption feature. The $180 thousand is classified as a debt discount, which is reflected as a reduction of the carrying value of long-term debt on the Company's consolidated balance sheet and is being amortized to interest expense over the term of the loan using the effective interest method. As a result of the June 2019 refinancing and entering into the 2019 Loan Agreement, the Company considered whether the previous debt was either extinguished or modified based on the difference in the cash flows of the previous and new debt. The Company determined that Hercules Loan Agreement, as amended by the First Amendment, was modified. Accordingly, the unamortized debt discount of the previous debt and newly incurred fees paid to the lender related to the 2019 Loan Agreement will be amortized to interest expense over the life of the new debt arrangement using the effective interest method.

The Company recognized aggregate interest expense under the Hercules Loan Agreement, as amended by the First Amendment, and the 2019 Loan Agreement of $1.8 million and $236 thousand during the twelve months ended December 31, 2019 and 2018, respectively. Interest expense includes $61 thousand and $355 thousand related to the accretion of the debt discount and the final payment for the year ended December 31, 2019, respectively. As of December 31, 2019, the unamortized debt discount was $317 thousand. The annual effective interest rate on the 2019 Loan Agreement is 10.9%. There were no principal payments due or paid under the Hercules Loan Agreement, as amended by the First Amendment, during the twelve months ended December 31, 2019. Principal payments begin in January 2022.

FFG Loan Agreements

Between September 2011 and March 2017, Arsanis GmbH entered into a series of funding agreements with FFG that provided for loans and grants to fund qualifying research and development expenditures of X4 GmbH on a project-by-project basis, as approved by FFG. Amounts due under the FFG loans bear interest at rates ranging from 0.75% to 2.00% per annum. Giving effect to the Settlement Agreement (as defined below), the loans matured at various dates between March 31, 2019 and March 31, 2021. Interest on amounts due under the loans is payable semi-annually in arrears, with all principal and remaining accrued interest due upon maturity.

On March 8, 2019, Arsanis, Merger Sub, X4 and Arsanis GmbH entered into a Settlement Agreement with FFG (the "Settlement Agreement") in respect to allegations by FFG in February 2019 that Arsanis and Arsanis GmbH breached certain reporting, performance and other obligations in connection with grants and loans made by FFG to Arsanis GmbH between September 2011 and March 2017 to fund qualifying research and development expenditures. Pursuant to the terms of the Settlement Agreement, in exchange for FFG's waiver of all claims against Arsanis and Arsanis GmbH (except for its claims for repayment of the loans and regular interest but including its waiver of claims for repayment of grants and interest exceeding regular interest), subject to compliance by Arsanis and Arsanis GmbH with the terms of the Settlement Agreement, Arsanis GmbH agreed to repay the outstanding loan principal (plus regular interest accrued thereon) on an accelerated payment schedule of three years instead of five years, with the final accelerated installment due and payable on June 30, 2021. The parties also agreed, among other things, that (i) the portion of such loans to be repaid in 2019 will be $2.9 million on the first business day following March 31, 2019, and such amount was paid on April 1, 2019, and (ii) until all of the loans have been repaid and subject to other terms specified in the Settlement Agreement, commencing April 30, 2019, a minimum cash balance equal to 70% of the then-outstanding principal amount of the loans will be maintained at X4 Pharmaceuticals (Austria) GmbH in an account held with an Austrian bank.

The Company recognized interest expense under the FFG agreement of $316 thousand for the year ended December 31, 2019. In September 2019, the Company repaid all amounts due under the FFG loans and recognized a loss on extinguishment of debt of $566 thousand, which was calculated as the difference between the reacquisition price of the borrowings under the FFG Loan Agreement and the carrying value of the debt at the time of the extinguishment.

As of December 31, 2019, future principal payments and the final payment due under the Company's loan agreements were as follows (in thousands):

Year Ending December 31	Total
2020	$ —
2021	—
2022	11,897
2023	8,103
Long-term debt	$ 20,000

9. Leases

Effective January 1, 2019, the Company adopted ASC 842 using the modified retrospective approach through a cumulative-effect adjustment and utilizing the effective date as its date of initial application, with prior periods unchanged and presented in accordance with the previous lease accounting guidance. Upon adoption, the Company recorded right-of-use assets of $2.0 million and lease liabilities of $2.5 million, of which $1.9 million was classified as non-current and $613 thousand as current. The difference between the value of the right-of-use assets and the lease liabilities related to $512 thousand of net deferred, accrued and prepaid rent that was reclassified against the right-of-use asset upon adoption of ASC 842 on January 1, 2019. The Company has lease agreements for its facilities in Cambridge, Massachusetts, which is the Company's global headquarters; Vienna, Austria, which is the Company's research and development center; Waltham, Massachusetts, which is the former headquarters of Arsanis that the Company has sublet to a third party; and Allston, Massachusetts, as further discussed below. There are no restrictions or financial covenants associated with any of the lease agreements.

Cambridge Lease— In August 2017, the Company entered into a non-cancellable operating lease agreement for office space of approximately 13,000 square feet in Cambridge, Massachusetts ("Cambridge Lease") which expires on July 31, 2022. The Cambridge Lease includes an annual fixed rent escalation clause and such rent escalations were included in the calculation of the right-of-use asset. The Company has the option to extend the lease for one period of 5 additional years. Base rent is approximately $832 thousand annually and the monthly rent expense is being recognized on a straight-line basis over the term of the lease as the Company amortizes the associated operating lease right-of-use asset. In addition to the base rent, the Company is also responsible

for its share of operating expenses, electricity and real estate taxes, in accordance with the terms of lease. These costs are not included in the determination of the leases' right-of-use operating assets or lease operating liabilities.

Waltham Lease— On March 13, 2019, as part of its Merger with Arsanis, the Company acquired a non-cancellable operating lease for approximately 6,000 square feet of office space in Waltham, Massachusetts ("Waltham Lease"). The Waltham Lease, as amended, commenced on January 1, 2019, and expires approximately 5 years from the commencement date. The base rent is approximately $263 thousand annually. In addition to the base rent, the Company is also responsible for its share of operating expenses, electricity and real estate taxes, which costs are not included in the determination of the leases' right-of-use assets or lease liabilities. The Company is subleasing the space to a third party for the duration of the lease. As a result, the Company has adjusted the value of the right-of-use asset to its estimated fair value, which represents future sublease income less costs to obtain sublease. The right-of-use asset is being amortized to rent expense over the 5 year term of the lease.

Vienna Austria Lease— On March 13, 2019, as part of its Merger with Arsanis, the Company acquired an operating lease for approximately 400 square meters of laboratory and office space in Vienna, Austria, (the "Vienna Austria Lease") which commenced on March 1, 2019, as amended, for a term of 2 years. The lease is cancellable by the Company upon three months' notice with no penalty. The annual base rent is approximately $154 thousand. The Company has classified this lease as a short-term lease as it is not reasonably certain that the Company with not terminate the lease within one year and, accordingly, has not recorded a right-of-use asset. Accordingly, rent expense is recorded on a straight-line basis as incurred over the term of the lease.

Allston Lease— On November 11, 2019, the Company entered into a lease agreement for approximately 28,000 square feet of office space currently under construction in a building located in Allston, Massachusetts. The office space is expected to replace the Company's current headquarters located in Cambridge, Massachusetts. The Company intends to move into the premises in mid-2020 upon the completion of construction. Monthly rent payments under the lease are expected to commence in May, 2020, reflecting a 180-day rent-free period following the execution of the Lease, subject to the timely completion of construction of the premises. Base rental payments will be approximately $1.0 million annually, plus certain operating expenses. The term of the lease will continue until November 2026, unless earlier terminated in accordance with the terms of the lease. The Company has the right to sublease the premises, subject to landlord consent. The Company also has the right to renew the lease for an additional five years at the then prevailing effective market rental rate. The Company is required to provide the landlord with a $1.1 million security deposit in the form of a letter of credit, which is classified as restricted cash.

For the Allston lease, the Company will participate in the construction of the office space and will incur construction costs to prepare the office space for its use, which will be partially reimbursed by the landlord. The Company has concluded that these construction costs generate and enhance the landlord's assets and, as such, costs that are not reimbursed will be classified as prepaid rent and then reclassified to the right-of-use asset on the lease commencement date, which is expected to occur once the landlord's asset is completed and available for additional leasehold improvements funded by the Company. Upon the lease commencement date, which had not yet occurred as of December 31, 2019, the Company will recognize the lease liability, which will reflect the future rent payments for the term of the lease discounted at the Company's collateralized borrowing rate, and the right-of-use asset, which will be measured as the lease liability plus the prepaid rent incurred to date.

As the Company's leases do not provide an implicit rate, the Company estimated the incremental borrowing rate in calculating the present value of the lease payments. The Company utilizes its incremental borrowing rates, which are the rates incurred to borrow on a collateralized basis over a similar term an amount equal to the lease payments in a similar economic environment.

The components of lease expense for the year ended December 31, 2019 were as follows (dollars in thousands):

Lease Cost	For the Year Ended December 31, 2019
Fixed operating lease cost	$ 827
Short-term lease costs	122
Total lease expense	$ 949
Other information	
Cash paid for amounts included in the measurement of lease liabilities:	
Operating cash flows from operating leases	$ 1,007
Leased assets obtained in exchange for new operating lease liabilities [1]	$ 484
Weighted-average remaining lease term—operating leases	3.0 years
Weighted-average discount rate—operating leases	9.0 %
Sublease income	$ 14

[1] Acquired in the Merger

Maturities of lease liabilities due under these lease agreements as of December 31, 2019 are as follows (in thousands):

Maturity of lease liabilities	Operating Leases
2020	$ 1,124
2021	1,098
2022	754
2023	263
Total lease payments	3,239
Less: interest	(423)
Total operating lease liabilities as of December 31, 2019	$ 2,816

The above table does not include future lease payments related to the Company's Allston lease for which a lease liability has not yet been recorded. The lease liability will be recorded upon the commencement date of the lease. Future lease payments upon the commencement date of the lease are expected to be approximately $0.6 million in 2020, $1.0 million in 2021, $1.0 million in 2022, $1.1 million in 2023, $1.1 million in 2024, $1.1 million in 2025 and $1.1 million in 2026. In addition, the Company expects to incur unreimbursed expenditures of $3.1 million related to the construction of the office space.

As of December 31, 2018, minimum future lease payments under non-cancellable operating leases for each of the following five years and a total thereafter were as follows (in thousands):

Year Ending December 31,	Operating Leases
2019	$ 810
2020	823
2021	835
2022	492
Total	$ 2,960

10. Commitment and Contingencies

Sponsored Research Agreement Commitments— In April 2017, the Company entered into a sponsored research agreement with a university pursuant to which the Company and the university are conducting a research program related to understanding

the mechanisms of failed long-term adaptive immunity in WHIM patients. Under the terms of the agreement, the Company agreed to provide funding for the research program of up to $499 thousand over a three years period. The agreement will remain in effect for three years, unless earlier terminated. The Company may terminate the agreement at any time upon at least 60 days' prior written notice. For the years ended December 31, 2019, 2018, and 2017, the Company incurred $166 thousand, $166 thousand and $111 thousand, respectively, of research and development expenses related to its payment obligations to the university under the agreement. As of December 31, 2019, the Company had non-cancelable purchase commitments under this agreement totaling $55 thousand committed in 2020.

In May 2019, the Company amended its agreement with a clinical research organization ("CRO") pursuant to which the Company and the CRO are conducting a global Phase 3 clinical trial of mavorixafor for the treatment of WHIM syndrome. The Company may terminate the agreement by providing 30 days' notice and if such termination occurred, the Company would incur early termination fees of up to $0.6 million based on a percentage of committed resources of the CRO as of the termination.

Indemnification Agreements— In the ordinary course of business, the Company may provide indemnification of varying scope and terms to vendors, lessors, business partners and other parties with respect to certain matters including, but not limited to, losses arising out of breach of such agreements or from intellectual property infringement claims made by third parties. In addition, the Company has entered into indemnification agreements with members of its board of directors and its executive officers that will require the Company to, among other things, indemnify them against certain liabilities that may arise by reason of their status or service as directors or officers. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. To date, the Company has not incurred any material costs as a result of such indemnification obligations. The Company is not currently aware of any indemnification claims and has not accrued any liabilities related to such obligations in its consolidated financial statements as of December 31, 2019 or December 31, 2018.

License Agreements— In February 2017, Arsanis entered into an option and license agreement with Adimab to obtain rights to certain RSV antibodies, which are being used in the "ASN500" program. The Company exercised this option for an up-front payment of $250 thousand. In July 2019, as further described in Note 16, the Company transferred the intellectual property related to the ASN500 program through an exclusive, worldwide license with a third party. The Company remains obligated to make payments to Adimab based on future clinical and regulatory milestones of up to approximately $25.0 million, as well as royalty payments on a product-by-product and country-by-country basis of a mid-single-digit percentage based on net sales of products based on certain RSV antibodies during the applicable term for such product in that country. The third party is required to fund any future payments that may become due to Adimab.

Legal Proceedings— The Company is not a party to any litigation and does not have contingency reserves established for any litigation liabilities. At each reporting date, the Company evaluates whether or not a potential loss amount or a potential range of loss is probable and reasonably estimable under the provisions of the authoritative guidance that addresses accounting for contingencies. The Company expenses as incurred the costs related to such legal proceedings.

11. Preferred and Common Stock Warrants

Prior to the Merger (see Note 1), the Company issued warrants for the purchase of its preferred stock and had classified these preferred stock warrants as a liability on its consolidated balance sheet as the warrants were deemed to be freestanding financial instruments that may have required the Company to transfer assets upon exercise. The liability associated with each of these warrants was initially recorded at fair value upon the issuance date of each warrant and was subsequently remeasured to fair value as a component of other income (expense), net in the consolidated statement of operations and comprehensive loss. Upon the closing of the Merger, pursuant to the Merger Agreement, all of the outstanding X4 preferred stock was converted to Arsanis common stock and the X4 preferred stock warrants converted to warrants for the purchase of Arsanis common stock. The Company assessed the features of the warrants and determined that they qualify for classification as permanent equity upon the closing of the Merger. Accordingly, the Company remeasured the warrants to fair value upon the closing of the Merger, which was $5.2 million at March 13, 2019, with $288 thousand of expense recorded during the three months ended March 31, 2019. Upon the closing of the Merger, the warrant liability was reclassified to additional paid-in capital.

In connection with its issuance of common stock in public offerings that closed on April 16, 2019 and November 29, 2019, the Company issued 3,900,000 Class A warrants, which are exercisable for shares of the Company's common stock, and 5,416,667

Class B warrants, which are exercisable for shares of the Company's common stock or prefunded warrants to purchase shares of the Company's common. The Class A warrants have an exercise price of $13.20 per warrant, expire on April 15, 2024 and were immediately exercisable upon issuance. The Class B warrants were immediately exercisable upon issuance, have an exercise price of $15.00 per warrant and expire on a date that is the earlier of (a) the date that is 30 calendar days from the date on which the Company issues a press release announcing top-line data from its Phase 3 clinical trial of mavorixafor for the treatment of patients with WHIM syndrome (or, if such date is not a business day, the next business day) and (b) November 28, 2024. In addition, in connection with the April 16, 2019 and November 29, 2019 offerings, the Company issued 2,130,000 and 1,750,000 prefunded warrants, respectively, for proceeds of $10.999 and $11.999 per share, respectively. Each of the prefunded warrants is exercisable into one share of the Company's common stock, has a remaining exercise price of $0.001 per share and was immediately exercisable upon issuance.

The following table provides a roll forward of outstanding warrants for the twelve month period ended December 31, 2019:

	Number of warrants		Weighted Average Exercise Price	Weighted Average Contractual Term (Years)
Outstanding and exercisable warrants to purchase preferred shares as of December 31, 2018	5,146,400	$	—	4.23
Conversion of warrants to purchase preferred shares to warrants for the purchase of common stock and adjusted for the Exchange Ratio and Reverse Stock Split	(4,657,350)			
Issuance of warrants for the purchase of common stock or prefunded warrants to purchase common stock	13,201,667		13.43	
Exercised	(33,846)		13.20	
Outstanding and exercisable as of December 31, 2019	13,656,871	$	13.68	4.59

As of December 31, 2019, the Company’s outstanding warrants to purchase shares of common stock consisted of the following:

Issuance Date	Number of Shares of Common Stock Issuable		Exercise Price	Classification	Expiration Date
August 14, 2015	81,228	$	21.78	Equity	August 14, 2020
August 21, 2015	69,603	$	21.78	Equity	August 21, 2020
October 25, 2016	5,155	$	19.78	Equity	October 24, 2026
November 1, 2017	130,609	$	19.78	Equity	October 31, 2020
November 17, 2017	8,442	$	19.78	Equity	November 16, 2020
December 4, 2017	5,661	$	19.78	Equity	December 3, 2020
December 28, 2017	6,925	$	19.78	Equity	December 27, 2020
December 28, 2017	115,916	$	19.78	Equity	December 28, 2027
September 12, 2018	25,275	$	19.78	Equity	September 12, 2021
September 12, 2018	20,220	$	19.78	Equity	September 12, 2028
October 19, 2018	20,016	$	19.78	Equity	October 19, 2028
March 13, 2019	5,000	$	19.80	Equity	March 12, 2029
April 16, 2019	3,866,154	$	13.20	Equity	April 15, 2024
April 16, 2019	2,130,000	$	11.00	Equity	n/a
November 29, 2019	5,416,667	$	15.00	Equity	November 28, 2024
November 29, 2019	1,750,000	$	12.00	Equity	n/a
	13,656,871				

As of December 31, 2018, the Company's outstanding warrants to purchase shares of preferred stock (which converted into warrants to purchase common stock upon close of the Merger) consisted of the following (not adjusted for the Reverse Stock Split or Exchange Ratio):

December 31, 2018

Issuance Date	Number of Shares of Preferred Stock Issuable	Exercise Price	Exercisable for	Classification	Expiration Date
August 14, 2015	854,785	$ 2.07	Series A	Liability	August 14, 2020
August 21, 2015	732,453	$ 2.07	Series A	Liability	August 21, 2020
October 25, 2016	54,256	$ 1.88	Series A	Liability	October 24, 2026
November 1, 2017	1,374,435	$ 1.88	Series B	Liability	October 31, 2020
November 17, 2017	88,845	$ 1.88	Series B	Liability	November 16, 2020
December 4, 2017	59,576	$ 1.88	Series B	Liability	December 3, 2020
December 28, 2017	72,875	$ 1.88	Series B	Liability	December 27, 2020
December 28, 2017	1,219,815	$ 1.88	Series B	Liability	December 28, 2027
September 12, 2018	265,957	$ 1.88	Series B	Liability	September 12, 2021
September 12, 2018	212,765	$ 1.88	Series B	Liability	September 12, 2028
October 19, 2018	210,638	$ 1.88	Series B	Liability	October 19, 2028
	5,146,400				

12. Common Stock, Redeemable Common Stock, and Convertible Preferred Stock (converted to Common Stock)

Common Stock— As of December 31, 2019 and December 31, 2018, the Company's Certificate of Incorporation, as amended and restated, authorized the Company to issue 33,333,333 shares and 11,070,776, shares, respectively, of $0.001 par value common stock. The voting, dividend and liquidation rights of the holders of the Company's common stock are subject to and qualified by the rights, powers and preferences of the holders of the preferred stock. Each share of common stock entitles the holder to one vote on all matters submitted to a vote of the Company's stockholders. Common stockholders are entitled to receive dividends, as may be declared by the board of directors, if any. No cash dividends have been declared or paid to date.

On April 12, 2019, the Company entered into an underwriting agreement with Cowen and Company, LLC and Stifel, Nicolaus & Company, Incorporated, as representatives of the several underwriters named therein pursuant to which it sold 5,670,000 shares of common stock and, in lieu of common stock, prefunded warrants to purchase 2,130,000 shares of common stock, and accompanying Class A warrants to purchase 3,900,000 shares of its common stock. The common stock was issued at a price to the public of $11.00 per share and accompanying Class A warrant and the prefunded warrants were issued at a price of $10.999 per prefunded warrant and accompanying Class A warrant. The Class A warrants have an exercise price of $13.20, will expire 5 years from the date of issuance, and are immediately exercisable with certain restrictions. The gross proceeds from the offering, which closed on April 16, 2019, were $85.8 million before deducting underwriting discounts and offering expenses.

On November 26, 2019, the Company entered into an underwriting agreement with Cowen and Company, LLC and Stifel, Nicolaus & Company, Incorporated, as representatives of the several underwriters named therein pursuant to which it sold 3,666,667 shares of common stock and, in lieu of common stock, prefunded warrants to purchase 1,750,000 shares of common stock, and accompanying Class B warrants to purchase 5,416,667 shares of its common stock or prefunded warrants to purchase shares of common stock. The common stock was issued at a price to the public of $12.00 per share and accompanying Class B warrant and the prefunded warrants were issued at a price of $11.999 per prefunded warrant and accompanying Class B warrant. The Class B warrants have an exercise price of $15.00 per warrant, which includes a down-round contingent price adjustment feature; expire on a date that is the earlier of (a) the date that is 30 calendar days from the date on which the Company issues a press release announcing top-line data from its Phase 3 clinical trial of mavorixafor for the treatment of patients with WHIM syndrome (or, if such date is not a business day, the next business day) and (b) November 28, 2024; and were immediately exercisable upon issuance. The gross proceeds from the offering, which closed on November 29, 2019, were $65.0 million before deducting underwriting discounts and offering expenses.

For each of the common stock offerings, the Company evaluated the Class A, Class B and prefunded warrants for liability or equity classification in accordance with the provisions of ASC 480, *Distinguishing Liabilities from Equity*, and ASC 815-40, *Derivatives and Hedging*, and determined that equity treatment was appropriate because neither the Class A, Class B or prefunded warrants meet the definition of a liability.

Redeemable Common Stock— Pursuant to the requirements of the July 2014 license agreement with Genzyme (see Note 4), in August 2015, the Company issued to Genzyme for no additional consideration 107,371 shares of common stock, which had an aggregate fair value of $734 on the date of issuance. Genzyme had the right to require the Company to repurchase all, but not less than all, of these shares of common stock at any time during the term of the license agreement for a price of $0.001 per share. Because of this redemption feature, the shares of common stock issued to Genzyme were classified outside of stockholders' deficit on the consolidated balance sheets. As a result of the Merger, these shares were exchanged for common stock.

Convertible Preferred Stock (converted to Common Stock)— The Company has issued Series Seed convertible preferred stock (the "Series Seed preferred stock"), Series A convertible preferred stock (the "Series A preferred stock") and Series B convertible preferred stock (the "Series B preferred stock"). As of December 31, 2019 and December 31, 2018, the Company's Certificate of Incorporation, as amended and restated, authorized the Company to issue a total of 10,000,000 shares and 59,413,523 shares, respectively, of preferred stock, with a par value of $0.001 per share.

The holders of Preferred Stock have liquidation rights in the event of a deemed liquidation that, in certain situations, are not solely within the control of the Company. Therefore, the Preferred Stock is classified outside of stockholders' deficit on the consolidated balance sheet.

As of December 31, 2019, there was no preferred stock outstanding. As of December 31, 2018, the preferred stock consisted of the following:

	December 31, 2018				
	Preferred Stock Designated	Preferred Stock Issued and Outstanding	Carrying Value	Liquidation Preference	Common Stock Issuable Upon Conversion [1]
Series Seed preferred stock	2,313,523	1,516,136	$ 1,310,000	$ 1,444,000	143,630
Series A preferred stock	22,000,000	19,946,862	32,480,000	47,624,000	1,895,610
Series B preferred stock	25,100,000	18,616,569	30,885,000	34,999,000	1,769,190
	49,413,523	40,079,567	$ 64,675,000	$ 84,067,000	3,808,430

[1] Adjusted to reflect Reverse Stock Split and Exchange Ratio.

13. Stock-Based Compensation

Summary of Plans— Upon completion of the Merger with Arsanis on March 13, 2019, X4's 2015 Employee, Director and Consultant Equity Incentive Plan, as amended (the "2015 Plan"), Arsanis' 2017 Equity Incentive Plan (the "2017 Plan") and Arsanis' 2017 Employee Stock Purchase Plan (the "ESPP") were assumed by the Company. In June 2019, the Company adopted the 2019 Inducement Equity Incentive Plan (the "2019 Plan"). These plans are administered by the Board of Directors or, at the discretion of the Board of Directors, by a committee of the Board of Directors. The exercise prices, vesting and other restrictions are determined at the discretion of the Board of Directors, or its committee if so delegated, except that the exercise price per share of stock options may not be less than 100% of the fair market value of the share of common stock on the date of grant and the term of the stock option may not be greater than ten years. Incentive stock options granted to employees and restricted stock awards granted to employees, officers, members of the board of directors, advisors, and consultants of the Company typically vest over four years. Non-statutory options granted to employees, officers, members of the Board of Directors, advisors, and consultants of the Company typically vest over three or four years. Shares that are expired, terminated, surrendered or canceled under the Plans without having been fully exercised will be available for future awards. In addition, shares of common stock that are tendered to the Company by a participant to exercise an award are added to the number of shares of common stock available for the grant of awards.

2015 Employee, Director and Consultant Equity Incentive Plan— In 2015, the Board of Directors and shareholders of X4 adopted the 2015 Plan, which provided for the Company to grant incentive stock options or nonqualified stock options, restricted

stock awards and other stock-based awards to employees, directors and consultants of the Company. Each stock option outstanding under the 2015 Plan at the effective time of the Merger was automatically converted into a stock option exercisable for a number of shares of the Company's common stock calculated based on the Exchange Ratio and the exercise price per share of such outstanding stock option.

The total number of shares of common stock that may be issued under the 2015 Plan is 969,340 shares, adjusted for the Exchange Ratio and Reverse Stock Split. Shares that are expired, forfeited, canceled or otherwise terminated without having been fully exercised will be available for future grant under the 2015 Plan. As of December 31, 2019, approximately 100,000 shares were available for future issuance under the 2015 Plan. In addition, shares of common stock that are tendered to the Company by a participant to exercise an award are added to the number of shares of common stock available for future grants.

2017 Equity Incentive Plan— In 2017, the Board of Directors and shareholders of Arsanis adopted the 2017 Plan, which provided for the Company to grant incentive stock options, non-qualified options, stock appreciation rights, restricted stock awards, restricted stock units and other stock-based awards. Incentive stock options may be granted only to the Company's employees, including officers and directors who are also employees. Awards other than incentive stock options may be granted to employees, officers, members of the board of directors, advisors and consultants of the Company. The number of shares of common stock reserved for issuance under this plan will automatically increase on January 1 of each year, through January 1, 2027, in an amount equal to the lowest of 170,915 shares of the Company's common stock (as adjusted for the Reverse Stock Split), 4.0% of the number of shares of the Company's common stock outstanding on January 1 of each year and an amount determined by the Company's Board of Directors. As of December 31, 2019, approximately 20,000 shares were available for future issuance under the 2017 Plan.

Employee Stock Purchase Plan— In 2017, the Board of Directors and shareholders of Arsanis adopted the ESPP, which provides participating employees with the opportunity to purchase shares of the Company's common stock at defined purchase prices over six-month offering periods. For the twelve months ended December 31, 2019, no shares of common stock were issued under the ESPP.

2019 Inducement Equity Incentive Plan— On June 17, 2019, the Board of Directors approved the adoption of the 2019 Plan, which provides for the Company to grant nonqualified stock options, restricted stock awards and other stock-based awards to new employees of the Company. Awards issued from the 2019 Plan are intended to be material inducements to each employee's acceptance of employment with the Company in accordance with Nasdaq Listing Rule 5635(c)(4). The total number of shares of common stock that may be issued under the 2019 Plan is 400,000 shares. Shares that are expired, forfeited, canceled or otherwise terminated without having been fully exercised will be available for future grant under the 2019 Plan. In addition, shares of common stock that are tendered to the Company by a participant to exercise an award are added to the number of shares of common stock available for future grants. As of December 31, 2019, approximately 250,000 shares were available for future issuance under the 2019 Plan.

Stock Option Valuation— The following table presents, on a weighted average basis, the assumptions used in the Black-Scholes option-pricing model to determine the grant-date fair value of stock options granted to employees, directors and non- employees:

	Year Ended December 31,		
	2019	2018	2017
Risk-free interest rate	2.0 %	2.8 %	2.1 %
Expected term (in years)	5.99	5.94	6.06
Expected volatility	88.5 %	86.0 %	77.3 %
Expected dividend yield	0 %	0 %	0 %

Stock Options

The following table summarizes the Company's stock option activity for the twelve months ended December 31, 2019:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Contractual Term (Years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2018	797,931	$ 8.29	8.42	$ 6,486
Assumed as part of Merger with Arsanis	271,230	62.60		
Granted	589,110	14.69		
Exercised	(51,617)	7.05		
Forfeited	(309,625)	31.55		
Outstanding as of December 31, 2019	1,297,029	$ 17.05	8.41	$ 1,286
Exercisable as of December 31, 2019	495,740	$ 21.66	7.21	$ 952
Vested and expected to vest as of December 31, 2019	1,122,542	$ 17.15	8.32	$ 1,245

The aggregate intrinsic value of options is calculated as the difference between the exercise price of the stock options and the fair value of the Company's common stock for those options that had exercise prices lower than the fair value of the Company's common stock. The aggregate intrinsic value of stock options exercised during the twelve months ended December 31, 2019 was $363 thousand. The intrinsic value of options exercised during the years ended December 31, 2018 and 2017 was not significant. The weighted average grant-date fair value per share of stock options granted during the years ended December 31, 2019, 2018 and 2017 was $10.78, $7.05, and $4.74, respectively.

Restricted Stock Units— During the twelve months ended December 31, 2019, the Company granted 116,689 restricted stock units to employees at a weighted average grant date fair value of $14.75 per share. The restricted stock units vest 25% annually on the grant anniversary over four years and had a grant date fair value of $1.7 million, which will be recognized as stock-based compensation expense, net of estimated forfeitures, over the vesting period.

The following table summarizes the Company's restricted stock activity for the twelve months ended December 31, 2019:

	Number of Shares
Unvested at December 31, 2018	—
Granted	116,689
Vested	—
Forfeited	(14,916)
Unvested at December 31, 2019	101,773

Stock-Based Compensation— Effective January 1, 2019, the Company adopted ASU 2018-7 and no longer remeasures the fair value of equity awards granted to non-employees at each reporting period end (see Note 2).

As of December 31, 2019, total unrecognized compensation expense related to unvested stock options and restricted stock units was $6.4 million, which is expected to be recognized over a weighted average period of 3.1 years.

Stock-based compensation expense was classified in the consolidated statements of operations as follows:

	Year Ended December 31,		
	2019	2018	2017
Research and development expense	$ 909	$ 258	$ 126
General and administrative expense	1,141	501	366
Total stock-based compensation	$ 2,050	$ 759	$ 492

14. Income Taxes

During the years ended December 31, 2019, 2018, and 2017, the Company recorded no income tax benefits for the net operating losses incurred and research and development credits generated due to the uncertainty of realizing a benefit from those items. The Company's losses before income taxes were generated in the United States and Austria.

Loss before the provision for income taxes for the years ended December 31, 2019, 2018 and 2017 consisted of the following:

	Year Ended December 31,		
(in thousands)	2019	2018	2017
United States	$ (52,314)	$ (33,285)	$ (21,994)
Foreign (Austria)	(493)	—	—
	$ (52,807)	$ (33,285)	$ (21,994)

A reconciliation of the U.S. federal statutory income tax rate to the Company's effective income tax rate is as follows:

	Year Ended December 31,		
	2019	2018	2017
U.S. federal statutory income tax rate	(21.0)%	(21.0)%	(34.0)%
State income taxes, net of federal benefit	(5.8)	(6.2)	(8.4)
Foreign rate differential	(0.1)	—	—
Research and development tax credits	(1.1)	(3.7)	(1.8)
Change in fair value of preferred stock warrant liability	0.1	2.4	(2.2)
Other permanent differences	1.4	0.9	0.5
Remeasurement of deferred taxes due to tax reform	—	—	27.8
Change in deferred tax asset valuation allowance	26.8	27.8	18.1
Other	(0.3)	(0.2)	—
Effective income tax rate	— %	— %	— %

Net deferred tax assets as of December 31, 2019 and 2018 consisted of the following:

	December 31,	
(in thousands)	2019	2018
Net operating loss carryforwards	$ 65,487	$ 15,482
Research and development tax credit carryforwards	3,654	2,710
Capitalized research and development expenses	2,626	2,837
Capitalized license fees	—	221
Accrual-to-cash basis conversion	—	1,367
Lease liabilities	765	—
Other	1,413	180
Total deferred tax assets	73,945	22,797
Valuation allowance	(73,410)	(22,797)
Deferred tax assets, net of valuation allowance	$ 535	$ —
Right of use assets	535	—
Total deferred tax liabilities	$ 535	$ —
Total deferred tax assets, net	$ —	$ —

As of December 31, 2019, the Company had U.S. federal and state net operating loss carryforwards of $181.8 million and $177.4 million, respectively, which may be available to offset future taxable income and begin to expire in 2031 and 2035, respectively, and of which $127.7 million related to U.S federal income taxes do not expire but are limited in their usage to an annual deduction equal to 80% of annual taxable income. In addition, as of December 31, 2019 the Company had foreign net operating loss carryforward of $64.4 million, which do not expire. U.S. federal and state net operating loss carryforwards include $75 million and $70 million, respectively, of carryforwards acquired in the Merger with Arsanis, the utilization of which may be subject to limitations as described below. As of December 31, 2019, the Company also had U.S. federal and state research and development tax credit carryforwards of $3.1 million and $0.6 million, respectively, which may be available to offset future tax liabilities and each begin to expire in 2032 and 2030, respectively. As of December 31, 2019, uncertain tax position reserves recorded were $0.2 million for U.S. federal and state research and development tax credits.

Utilization of the U.S. net operating loss carryforwards and research and development tax credit carryforwards may be subject to a substantial annual limitation under Section 382 of the Internal Revenue Code of 1986 due to ownership changes that have occurred previously or that could occur in the future. These ownership changes may limit the amount of carryforwards that can be utilized annually to offset future taxable income. In general, an ownership change, as defined by Section 382, results from transactions increasing the ownership of certain shareholders or public groups in the stock of a corporation by more than 50% over a three-year period. The Company has not conducted a study to assess whether a change of control has occurred or whether there have been multiple changes of control since inception due to the significant complexity and cost associated with such a study. If the Company has experienced a change of control, as defined by Section 382, at any time since inception, utilization of the U.S. net operating loss carryforwards or research and development tax credit carryforwards would be subject to an annual limitation under Section 382, which is determined by first multiplying the value of the Company's stock at the time of the ownership change by the applicable long-term tax-exempt rate, and then could be subject to additional adjustments, as required. Any limitation may result in expiration of a portion of the net operating loss carryforwards or research and development tax credit carryforwards before utilization.

Each period, the Company evaluates the positive and negative evidence bearing upon its ability to realize the deferred tax assets. Management has considered the Company's history of cumulative net losses incurred since inception and its lack of commercialization of any products or generation of any revenue from product sales since inception and has concluded that it is more likely than not that the Company will not realize the benefits of its deferred tax assets. Accordingly, a full valuation allowance has been established against the deferred tax assets as of December 31, 2019, 2018 and 2017.

Changes in the valuation allowance for deferred tax assets during the years ended December 31, 2019, 2018 and 2017 related primarily to the increases in net operating loss carryforwards and research and development tax credit carryforwards and were as follows:

	Year Ended December 31,		
(in thousands)	**2019**	**2018**	**2017**
Valuation allowance, beginning of year	$ (22,797)	$ (13,546)	$ (9,738)
Increases recorded to income tax provision	(14,485)	(9,251)	(3,808)
Acquisition of business	(36,128)	—	—
Valuation allowance, end of year	$ (73,410)	$ (22,797)	$ (13,546)

The Company's U.S. federal and state income tax returns are generally subject to tax examinations for the tax years ended December 31, 2016 through December 31, 2019. There are currently no pending income tax examinations. To the extent the Company has tax attribute carryforwards, the tax years in which the attribute was generated may still be adjusted upon examination by the Internal Revenue Service and state tax authorities to the extent utilized in a future period. The Company's policy is to record interest and penalties related to income taxes as part of its income tax provision.

15. Net Loss per Share

Basic and diluted net loss per share attributable to common stockholders was calculated as follow:

(in thousands, except per share data)	Year Ended December 31, 2019	2018	2017
Numerator:			
Net loss	$ (52,807)	$ (33,285)	$ (21,994)
Accruing dividends on Series A convertible preferred stock	(592)	(3,000)	(3,000)
Adjustment to accumulated deficit in connection with repurchase of Series Seed convertible preferred stock	—	(22)	—
Net loss attributable to common stockholders	$ (53,399)	$ (36,307)	$ (24,994)
Denominator:			
Weighted average shares of common stock—basic and diluted	11,530	459	458
Net loss per share attributable to common stockholders— basic and diluted	$ (4.63)	$ (79.15)	$ (54.58)

The Company has included 107,371 shares of redeemable common stock in its computation of basic and diluted weighted average shares of common stock outstanding for the years ended December 31, 2019, 2018 and 2017 as this class of stock participates in losses similarly to other common stockholders. Basic and diluted weighted average shares of common stock outstanding for the year ended December 31, 2019 also includes the weighted average effect of 2,130,000 and 1,750,000 prefunded warrants for the purchase of shares of common stock, which were issued in April 2019 and November 2019, respectively, and for which the remaining unfunded exercise price is less than $0.001 per share.

The Company's potentially dilutive securities included outstanding stock options, convertible preferred stock, and warrants to purchase shares of convertible preferred stock for the year ended December 31, 2018 and included outstanding stock options and warrants to purchase common stock for the year ended December 31, 2019. These potentially dilutive securities have been excluded from the computation of diluted net loss per share as the effect would be to reduce the net loss per share, and thus they are considered "anti-dilutive." Therefore, the weighted average number of common shares outstanding used to calculate both basic and diluted net loss per share attributable to common stockholders is the same. The Company excluded the following potential shares of common stock, presented based on amounts outstanding at each period end and adjusted for the Exchange Ratio and Reverse Stock Split, from the computation of diluted net loss per share attributable to common stockholders for the periods indicated because including them would have had an anti-dilutive effect:

	Year Ended December 31, 2019	2018	2017
Options to purchase common stock and unvested restricted stock units	1,398,802	798,311	474,282
Convertible preferred stock (as converted to common stock)	—	3,808,894	3,613,069
Warrants to purchase common stock (excluding prefunded warrants, which are included in basic shares outstanding)	9,776,871	489,079	423,567
	11,175,673	5,096,284	4,510,918

16. Loss on Transfer of Nonfinancial Assets

During the year ended December 31, 2019, the Company entered into contractual arrangements with two third parties that transferred the rights to develop and commercialize the programs underlying IPR&D intangible assets acquired in the Merger. As of December 31, 2019, all programs underlying IPR&D intangible assets acquired in the Merger were transferred to these third parties and the Company has no continuing involvement in any ongoing research and development activities associated with the programs. The Company concluded that these third parties are "non-customers" as the underlying development programs transferred to these third parties are focused on potential drug candidates that were not aligned with the Company's strategic focus and, therefore, are not an output of the Company's ordinary activities. Accordingly, the Company accounted for these transactions under ASC Topic 610-20, *Gains and Losses from the Derecognition of Nonfinancial Assets* ("ASC 610-20"). As a result of the transfer of control of the IPR&D projects to third parties, the Company derecognized the IPR&D intangible assets through a

charge to "loss on transfer of nonfinancial assets" in 2019. The $3.9 million loss includes the carrying value of the IPR&D of $4.9 million, partially offset by $1.0 million of non-refundable, upfront fees received from the third parties. In accordance with the contractual arrangements with these third parties, the Company is entitled to future fees that are contingent on the success of the third parties in advancing the IPR&D project toward commercialization. These future fees, if any, will be recognized in future periods as gains on the transfer of nonfinancial assets in accordance with ASC 610-20, which generally provides that the fees would be recognized when it is probable that a future reversal of the fees that would be material to cumulative fees recognized to date would not occur.