X4 PHARMACEUTICALS, INC.

2023 ANNUAL REPORT

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, 2023

or

☐ **ANNUAL 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.)
61 North Beacon Street, 4th Floor Boston, Massachusetts	**02134**
(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

Securities registered pursuant to Section 12(g) of the Act: none

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes ☐ No ☒

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes ☐ No ☒

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (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 has filed a report on an attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. Yes ☐ No ☒

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☐

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to § 240.10D-1(b). ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). ☐

On June 30, 2023, the aggregate market value of the registrant's voting common stock held by non-affiliates of the registrant was approximately $318 million based upon the closing sale price on the Nasdaq Capital Market reported on June 30, 2023. In determining the market value of non-affiliate common stock, shares of the registrant's common stock beneficially owned by officers, directors and affiliates have been excluded. This determination of affiliate status is not necessarily a conclusive determination for other purposes.

Independent Registered Public Accounting Firm	PricewaterhouseCoopers LLP	Boston, Massachusetts, US	Firm ID	238

As of March 18, 2024, there were 167,937,781 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, (the "2024 Proxy Statement") for its 2024 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, 2023, are incorporated by reference into Part III of this Annual Report on Form 10-K.

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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 (the "Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended (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 forward-looking statements are subject to a number of known and unknown risks, uncertainties and assumptions, including risks described in the section titled "Risk Factors" and elsewhere in this report, regarding, among other things:

- 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;
- the initiation, timing, progress and results of our current and future preclinical studies and clinical trials and related preparatory work and the period during which the results of the trials will become available, as well as our research and development programs;
- the potential benefits, including clinical utility, that may be derived from any of our 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;
- the benefits of U.S. Food and Drug Administration and European Commission designations, including, without limitation, Fast Track, orphan designation and Breakthrough Therapy;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our ability to attract and retain qualified employees and key personnel;
- our competitive position and the development of and projections relating to our competitors or our industry;
- 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 continue as a going concern;
- our plans to in-license, acquire, develop and commercialize additional product candidates;
- the impact of laws and regulations;
- our plans to identify additional product candidates with significant commercial potential that are consistent with our commercial objectives;
- our ability to raise additional capital;
- our strategies, prospects, plans, expectations or objectives; and
- other risks and uncertainties, including those listed under the section titled "Risk Factors" in this Annual Report.

You should refer to the section titled "Risk Factors" in this Annual Report 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.

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

PART I

ITEM 1. BUSINESS

Overview

We are a late clinical-stage biopharmaceutical company discovering and developing novel therapeutics for the treatment of rare diseases and those with limited treatment options, with a focus on conditions resulting from dysfunction of the immune system.

Our lead clinical candidate is mavorixafor, a small-molecule selective antagonist of chemokine receptor CXCR4, that is being developed as an oral, once-daily therapy. Due to its ability to increase the mobilization of mature, functional white blood cells into the bloodstream, we believe that mavorixafor has the potential to provide therapeutic benefit across a variety of chronic neutropenic disorders, including WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) syndrome, a rare, primary immunodeficiency.

We are currently seeking approval from the U.S. Food and Drug Administration ("FDA") for the use of oral, once-daily mavorixafor in the treatment of people aged 12 years and older with WHIM syndrome following the October 2023 acceptance of our New Drug Application ("NDA") by the FDA. The FDA has granted the NDA Priority Review, establishing a goal of six months review from the date of acceptance and assigning a Prescription Drug User Fee Act ("PDUFA") target action date of April 30, 2024. At this time, the FDA has notified us that they are not planning to hold an advisory committee meeting to review the filing. Due to mavorixafor's Rare Pediatric Disease designation in the U.S. for WHIM syndrome, should mavorixafor be approved, we are eligible to receive a Priority Review Voucher ("PRV"), which may be used to obtain Priority Review for a subsequent application or sold to another drug sponsor.

The NDA is supported by our successfully completed global, pivotal, Phase 3 clinical trial ("4WHIM") that evaluated the safety and efficacy of mavorixafor in 31 people with WHIM syndrome. The 4WHIM trial met its primary endpoint and a key secondary endpoint, demonstrating statistically significant increases in time above threshold for absolute neutrophil counts ("TAT-ANC") in patients treated with mavorixafor and time above threshold for absolute lymphocyte counts ("TAT-ALC") versus placebo. Additional data showed that mavorixafor treatment resulted in statistically significant reductions in annualized infection rates versus placebo and clinically meaningful reductions in both the severity and duration of infections versus placebo. Mavorixafor was generally well tolerated throughout the 52-week trial.

In anticipation of a potential second quarter 2024 U.S. launch of mavorixafor in WHIM syndrome, we have continued to build our go-to-market organization, with key hires across commercial and medical functions, increased interactions with key stakeholders and rare disease patient advocacy organizations, and launched a disease awareness campaign aiming to further the understanding of WHIM syndrome and educate patients and physicians on the importance and benefits of early diagnosis.

We are also advancing mavorixafor for the treatment of people with certain chronic neutropenic disorders following positive results from a Phase 1b clinical trial of a single dose of mavorixafor in people with idiopathic, cyclic, and congenital chronic neutropenia. We are conducting a Phase 2 clinical trial, evaluating the durability, safety, and tolerability of chronic dosing of once-daily oral mavorixafor with or without concurrent treatment with injectable granulocyte colony-stimulating factor ("G-CSF") in the same patient population. Preliminary results from the trial showed that the first three participants experienced clinically meaningful increases in absolute neutrophil counts ("ANC"). We expect to share further data from the Phase 2 trial in the second quarter of 2024. Concurrent with conducting this Phase 2 trial, we are advancing our plans for a Phase 3 trial of mavorixafor in people with certain chronic neutropenic disorders. This Phase 3 trial will be a global, randomized, placebo-controlled trial assessing the safety and efficacy of mavorixafor, with or without concomitant G-CSF, in people with idiopathic or congenital neutropenia. We expect that this Phase 3 trial will initiate in the second quarter of 2024.

We believe that successfully developing mavorixafor and providing new therapeutic options to individuals in the United States diagnosed with certain chronic neutropenic disorders has the potential to revolutionize the treatment landscape, which is principally served by injectable therapies that are frequently associated with treatment-limiting adverse events.

Our Focus

We have developed a pipeline of small-molecule, oral antagonists of the chemokine receptor CXCR4, or C-X-C receptor type 4. CXCR4 is a cell receptor that helps regulate the movement of immune cells within the body. CXCR4 receptor stimulation by its cognate ligand, CXCL12, has been shown to play a key role in the maturation and mobilization of white blood cells such as neutrophils, lymphocytes (including both B cells and T cells), and monocytes, into the bloodstream. Because antagonism of the CXCR4 receptor has been shown to increase the trafficking of white blood cells, we believe that therapeutic inhibition of the

CXCR4/CXCL12 axis holds the potential to benefit people with a wide variety of diseases where there remain significant unmet needs, including chronic neutropenic disorders and certain types of cancer.

Chronic neutropenic disorders are rare blood conditions where people of all ages experience low levels of neutrophils and tend to be at greater risk of developing infections and certain types of cancer. Depending on the levels of circulating neutrophils in the blood, neutropenia can be categorized as mild, moderate, or severe.

We are currently focused on advancing our lead clinical candidate, mavorixafor, for the treatment of a number of chronic neutropenia indications, including WHIM syndrome, while also pursuing partnership opportunities to further advance our previous work in oncology indications.

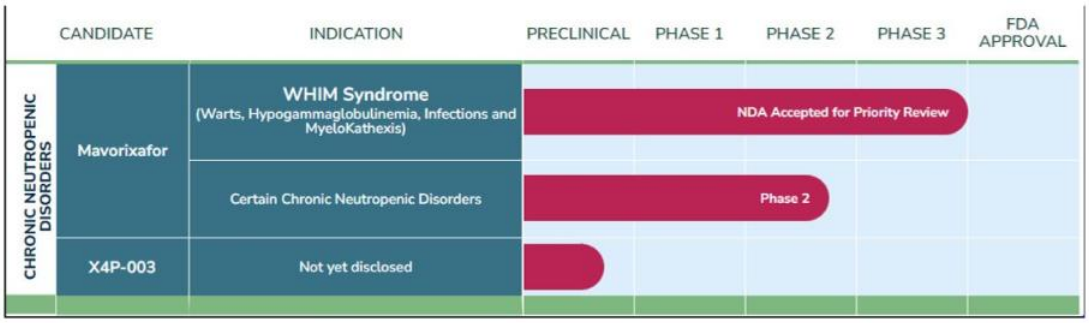
Our Pipeline

Our deep understanding of the biology of the CXCR4 pathway has enabled us to discover and develop multiple small-molecule CXCR4 antagonists. To date, we have advanced our lead candidate, mavorixafor, into late-stage clinical development. Mavorixafor is an orally available, small-molecule CXCR4 antagonist designed to facilitate the mobilization of white blood cells from the bone marrow into the blood, to increase levels of circulating neutrophils, lymphocytes, and monocytes, and to improve immune system function.

To date, more than 350 subjects in clinical trials have been dosed with mavorixafor, with a favorable tolerability profile observed. In these trials, we have observed drug exposure levels, a 22-hour half-life, and bioavailability of mavorixafor to support once-daily oral dosing, which we believe would provide convenient dosing and facilitate patient compliance for chronic or life-long use, if approved. The manufacturing process for mavorixafor utilizes well established, small-molecule chemistry. The commercial product, if approved, can be supported by specialty pharmacy distribution.

We have successfully advanced mavorixafor through a pivotal, Phase 3 clinical trial, referred to as the 4WHIM trial, in people with WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) syndrome, a rare, combined primary immunodeficiency. We have also completed a Phase 1b clinical trial of a single dose of mavorixafor in people with congenital, idiopathic, or cyclic neutropenia and we are now conducting a Phase 2 clinical trial evaluating the durability, safety, and tolerability of chronic dosing of once-daily oral mavorixafor with or without concurrent treatment with injectable G-CSF in the same patient population.

We have two pre-clinical candidates: X4P-003, a second-generation CXCR4 antagonist designed to have enhanced properties relative to mavorixafor, potentially enabling broader opportunities in CXCR4-dependent disorders and primary immunodeficiencies; and X4P-002, a CXCR4 antagonist with a unique distribution profile and a demonstrated ability to cross the blood-brain barrier.



About WHIM Syndrome

WHIM syndrome is both a rare, combined immunodeficiency and a congenital neutropenic disorder in which the body's immune system does not function properly and has trouble fighting infections. In many patients, WHIM is caused by "gain of function" variations in the single gene that encodes for the CXCR4 receptor. 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 most WHIM patients, however, a genetic variation in the receptor gene 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" signal and retention of white blood cells in the bone marrow where they are produced, leading to the chronic peripheral neutropenia, lymphopenia, and monocytopenia that are the clinical hallmarks of WHIM syndrome.

Genetic testing is typically used to diagnose WHIM syndrome to confirm the presence of a genetic variation in the CXCR4 receptor. The diagnosis of WHIM syndrome may occur at any age: about one-half of reported patients are diagnosed as children, primarily before of at the age of 18 years, with the other half diagnosed as adults, mostly between 18 and 40 years of age.

WHIM syndrome is named for its four common manifestations, although not all patients experience all symptoms, and not all symptoms are required for a diagnosis: **W**arts, related to infection with the Human Papilloma Virus (HPV), **H**ypogammaglobulinemia, a condition of low immunoglobulin ("IG") levels, **I**nfections, including both bacterial and fungal infections, and **M**yelokathexis, a hyper-dense population of immune cells in the bone marrow. These conditions reduce the body's ability to achieve a healthy immune response. Left untreated, those with WHIM syndrome may experience debilitating and life-threatening complications, including an increased cancer risk, irreversible end-organ damage, and/or sepsis.

The incidence and prevalence of WHIM syndrome are not well established. We believe that this is due to the relatively recent elucidation 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 disease-modifying treatments. Based on a preliminary U.S. market research study sponsored by us and conducted by a third-party research firm, we believe that the prevalence of WHIM syndrome worldwide is significantly higher than previous registries suggest.

- 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 identified to participate in this study reported more than 1,700 patients in the United States with genetically confirmed or highly probable WHIM syndrome.

In addition, we have also completed a study using artificial intelligence, interrogating a database of approximately 300 million American lives that included up to 10 years of insurance claims on diagnoses, drug treatments, procedures, and treatment pathways. This study suggests that there may be as many as 3,700 WHIM patients in the United States based on the WHIM-like phenotypes described.

The first CXCR4 genetic variation determined to cause WHIM syndrome was identified in 2003. Since then, several CXCR4 variations have been identified as "gain of function" variations causing WHIM syndrome. Our research has subsequently identified a number of new genetic variations, among them a new missense variation, called D84H, that is relatively frequent in the general population. The D84H mutation is the first mutation to be identified outside of the C-terminus of the CXCR4 receptor showing gain-of-function signaling and WHIM disease phenotype. We believe that the frequency of the D84H variation, as derived from broad population genomic databases, further supports our current WHIM prevalence estimate. Our research into additional WHIM-causing genetic variations is ongoing and we are continuing to identify novel pathogenic variants, further expanding our understanding of the clinical phenotype of people with WHIM syndrome.

In partnership with Invitae, a genetic information company, we sponsor a no-charge genetic testing and counseling program called PATH4WARD for individuals who may carry a genetic variation known to be associated with chronic neutropenia or primary immunodeficiency disorders ("PIDs"), including WHIM syndrome. The genetic panel looks at more than 550 genes known to be associated with PIDs; to date, the program has proven helpful not only in diagnosing WHIM, but also providing a better understanding of novel genetic variants causing PIDs and assisting participant enrollment in X4-sponsored clinical trials.

We continue to increase awareness of WHIM syndrome among patients, physicians, and their support systems through our partnerships with key patient foundations, including the Jeffrey Modell Foundation, International Patient Organisation for Primary Immunodeficiencies, and Immune Deficiency Foundation.

We have also deployed of a field force of Medical Science Liaisons ("MSLs") and Patient Diagnostic Liaisons ("PDLs") in the United States to further drive education and awareness of WHIM syndrome and its diagnosis. Upon obtaining approval of our NDA by the FDA, we plan to deploy a field sales force who will provide information about our approved drug product to health care providers who are known to or potentially could have WHIM patients under their care.

Limited Current Treatment Landscape for WHIM Syndrome

Currently, there are no approved therapies for the treatment of WHIM syndrome and care is limited to the treatment of the syndrome's different symptoms, mainly the prevention and management of infections. None of these symptomatic treatments, however, address the underlying cause of this multi-faceted disease. Current symptoms and their treatment 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.
- Hypogammaglobulinemia: Intravenous or subcutaneous IG administration, referred to as IVIG ("IVIG") or SCIG ("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 white blood cells 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 are known to 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 (damaged lung airways). Patients are sometimes given granulocyte-colony stimulating factor ("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. 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 also sometimes used to treat the myelokathexis characteristic of WHIM syndrome to try to increase the number of neutrophils in the peripheral blood, but G-CSF has no effect on lymphocytes and other types of white blood cells.

While the costs of managing the chronic impact of WHIM syndrome are unknown, the per-patient cost of treating primary immunodeficiencies that are similar to WHIM syndrome, based on drug costs alone, exceeds $100,000 per year in the United States for 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 genetic variations to the CXCR4 receptor, which is the established cause of WHIM syndrome.

Our approach to treating WHIM syndrome involves addressing the underlying cause of the disease by blocking CXCR4 signaling using mavorixafor, which has been shown to bind to the CXCR4 receptor in a manner that inhibits the receptor from being stimulated by CXCL12, enabling white blood cells to properly mature and release into the bloodstream and improving immune cell numbers and function.

Clinical Development of Mavorixafor in WHIM Syndrome

In January 2017, we initiated a Phase 2 clinical trial of mavorixafor for the treatment of people 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 ("IND") application that we submitted to the U.S. Food and Drug Administration ("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 pivotal Phase 3 clinical trial. The secondary objective of the Phase 2 trial was to evaluate the potential efficacy of mavorixafor in people with WHIM syndrome by measuring biomarkers, specifically absolute neutrophil ("ANC") and lymphocyte ("ALC") counts, 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, participants 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 cells per microliter or a lymphocyte count equal to or less than 650 cells per microliter.

In the trial, participants received escalating doses of mavorixafor starting at 50 mg once daily to up to 400 mg once daily. Participants were dose-escalated from their starting dose based on an in-hospital 24-hour measurement of ANC and ALC above or below the pre-defined target thresholds of 600 cells per microliter and 1,000 cells per microliter, respectively.

We completed the dose-titration portion of the Phase 2 clinical trial in March 2018 and, based on the reported results, the Data Review Committee recommended the Phase 3 dose of 400 mg administered orally once daily. The choice of time above threshold for absolute neutrophil count ("TAT-ANC"), defined as the number of hours during which the absolute neutrophil count is raised above a 500 cells per microliter threshold, was selected as the primary endpoint of the Phase 3 clinical trial.

Following completion of the dose-titration portion of the Phase 2 clinical trial, participants were allowed to continue on study drug in a Phase 2 open-label extension trial. In June 2020, we announced the following positive data from the open-label extension portion of the Phase 2 clinical trial:

- Sustained, dose-dependent increases in white blood cells, ANC, and ALC were achieved; higher doses of mavorixafor were shown to increase the TAT-ANC at least 4.5-fold versus the TAT-ANC at lower doses.
- These long-term hematological improvements correlated with fewer infections and reduced numbers of cutaneous warts.
- A decreased yearly infection rate from 4.63 [95%CI 3.3,6.3] events in the 12 months prior to the trial, to 2.27 [95%CI 1.4, 3.5] events when treated with mavorixafor at higher doses once daily; notably, deeper reductions in yearly infection rates correlated with increased time on treatment.
- The participants with cutaneous warts on hands and/or feet at baseline achieved an average 75% reduction in the number of warts.
- Mavorixafor was well tolerated for the extended duration of up to more than two years without any attributable serious adverse effects.

In December 2021, we announced the following additional data from the Phase 2 open-label extension trial of mavorixafor in people with WHIM syndrome:

- Mavorixafor continued to show durable increases in neutrophils, lymphocytes, and monocytes, sustained improvements in infections and warts, and was well tolerated (median treatment duration = 148.4 weeks).
- Decreases in mean annualized infection rates correlated well with TAT-ANC.
- Standardized participant interviews revealed that long-term treatment with mavorixafor was well tolerated and continued to demonstrate beneficial treatment effects, including decreased frequency, severity, and duration of infections and fewer hospital/doctor visits.

In June 2019, we initiated 4WHIM, a pivotal, global, randomized, double-blind, placebo-controlled, multicenter Phase 3 clinical trial designed to evaluate the efficacy and safety of mavorixafor in people with genetically confirmed WHIM syndrome. Originally designed to enroll 18-28 patients, the trial achieved full enrollment in September 2021, with 31 participants aged 12 and older receiving either 200-400 mg mavorixafor or placebo orally once daily for 52 weeks; all participants then became eligible to receive treatment with mavorixafor in an open-label trial extension.

The primary endpoint of the 4WHIM trial was a clinically relevant reduction of duration of severe neutropenia as measured by the increase in TAT-ANC (500 cells per microliter) in peripheral blood. Secondary endpoints include time above threshold-absolute lymphocyte count (TAT-ALC) of ≥ 1,000 cells per microliter over a 24-hour period, a composite clinical efficacy endpoint for mavorixafor based on total infection score and total wart change score, total wart change score for mavorixafor based on central

blinded, independent review of 3 target skin regions, total infection score for mavorixafor based on number and severity of infections adjudicated by a blinded, independent adjudication committee; and a number of quality-of-life measurements and other exploratory endpoints.

In November 2022, we reported positive top-line results from the Phase 3 4WHIM trial:

- 4WHIM met its primary endpoint, with mavorixafor achieving clinical and statistical superiority over placebo ($P<0.0001$) when measuring the length of time that participants' ANC remained above a clinically meaningful threshold of 500 cells per microliter (severe neutropenia), over 24-hour periods at 4 time points throughout the 52-week trial.
- 4WHIM also met a key secondary endpoint, with mavorixafor achieving clinical and statistical superiority over placebo ($P<0.0001$) when measuring the length of time that participants' ALC remained above a clinically meaningful threshold of 1,000 cells per microliter (lymphopenia), over 24-hour periods at 4 time points throughout the 52-week trial.
- Mavorixafor was generally well tolerated in the trial, with no treatment-related serious adverse events reported and no discontinuations for safety events.

In the second quarter of 2023, we presented additional data and analysis of the secondary and exploratory endpoints of the 4WHIM trial. Additional data presented revealed that mavorixafor treatment also resulted in statistically significant reductions in annualized infection rates versus placebo and effected clinically meaningful reductions in the both the severity and duration of infections versus placebo in trial participants. More specifically, the additional data showed the following:

- **Rate of Infections:** In the trial, mavorixafor treatment resulted in a statistically significant reduction (~60%) in annualized infection rate versus placebo ($p<0.01$). In addition, the data showed that the reduction was greater with time on treatment, with participants on mavorixafor experiencing less than one infection per year versus 4.5 for those on placebo; during the second 6 months of the trial, the difference also achieved statistical significance ($p<0.005$).
- **Severity of Infections:** During the trial, 29% (5 of 17) of those on placebo experienced Grade 3 or higher infections, whereas only 7% (1 of 14) of those on mavorixafor experienced a Grade 3 or higher infection, equating to a 75% reduction in the number of individuals experiencing severe infections. Importantly, the single Grade 3 infection in the mavorixafor treatment arm occurred during the first 3 months of the trial; after 3 months of treatment, there were no serious infections in the mavorixafor-arm, whereas the frequency of severe infections in those on placebo remained unchanged over the 52-week trial period.
- **Duration of Infections:** In the trial, mavorixafor treatment reduced the total duration (in days) of infections by more than 70% as compared to placebo, with those on placebo experiencing a mean of 7 weeks (49.1 days) with infections over the 52-week trial period versus a mean of only 2 weeks (14.1 days) with infections for those treated with mavorixafor.
- **Other Infection Metrics:** Mavorixafor treatment resulted in a 40% lower total infection score, a metric that combines infection number and severity (LS mean [95% CI]: mavorixafor, 7.41 [1.64–13.19]; placebo, 12.27 [7.24–17.30]). Treatment with mavorixafor also resulted in reductions in upper and lower respiratory tract and skin infections compared with those on placebo; participants on placebo required greater medical intervention, with 10 of the 17 participants on placebo requiring treatment with antibiotics over the course of the study, versus 3 of the 14 receiving mavorixafor; and slight improvements in warts were demonstrated both in the mavorixafor and placebo arms (no difference between arms).
- **Safety and Tolerability:** As reported previously, mavorixafor was generally well tolerated in the 4WHIM trial, with no drug-related serious adverse events, no treatment limiting toxicities, and no discontinuations due to safety. Approximately 90% of participants in the trial continued on to receive mavorixafor treatment at the start of the ongoing open-label extension study.

In August 2023, we submitted to the FDA an NDA with the goal of obtaining approval for mavorixafor for the treatment of people in the United States, aged 12 and older, with WHIM syndrome. The FDA accepted the NDA in late October 2023 for priority review and established a PDUFA target action date of April 30, 2024.

Mavorixafor has received multiple special designations from global regulatory authorities in WHIM syndrome, including Breakthrough Therapy Designation, Fast Track Designation, and Rare Pediatric Designation in the United States, and orphan designation in both the United States and European Union. In addition, upon approval of an NDA, we are eligible to receive a Priority Review Voucher ("PRV") as a result of mavorixafor's Rare Pediatric Designation in the United States. If obtained, such PRV could potentially be sold to a third party.

About Chronic Neutropenic Disorders
Due to its demonstrated ability to durably elevate levels of circulating white blood cells across multiple clinical trials, we believe that mavorixafor may be useful in the treatment of people with a variety of chronic neutropenic disorders.

Chronic neutropenia is defined as periods lasting more than three months persistently or intermittently where there are abnormally low levels of neutrophils circulating in the blood, and may be idiopathic (of unknown origin), cyclic (episodes typically occurring every three weeks), or congenital (of genetic causation). Similar to WHIM syndrome, chronic neutropenia disorders are rare blood conditions similarly characterized by increased risks of infections and cancer due to abnormally low levels of neutrophils in the body. In all cases, the CXCL12/CXCR4 pathway is the key regulator of neutrophil release from the bone marrow.

The incidence and prevalence of chronic neutropenic disorders are not well established. In December 2022, we presented results from what we believe was the first study examining the prevalence of chronic neutropenia disorders (including idiopathic, cyclic, and congenital neutropenia) in the United States; we believe that determining the estimated projected prevalence of chronic neutropenic disorders is a key step to understanding the extent of existing unmet medical needs in this patient population.

- Using a retrospective analysis of a large U.S. claims database, the analysis included people with a diagnosis code for neutropenia during the calendar years 2018, 2019, and 2021 (the year 2020 was excluded from this analysis owing to anticipated reduced claims during the COVID-19 pandemic).
- People with a diagnostic, procedural, or product code for neutropenia resulting from secondary causes including chemotherapy, drug exposure, infection, solid organ transplantation, myelodysplastic syndrome, and end-stage renal disease within 24-month period prior to selection were excluded.
- A 13- to 24-month look back period prior to index date was used to confirm chronic status.
- The analysis used longitudinal prescription data and office-based claims data from an IQVIA claims database that included 93% of retail prescription claims, 77% of mail-in prescription claims, and had more than 1.5 billion office-based claims per year.
- This retrospective analysis projected that in 2021, up to 48,000 people in the United States were living with a diagnosis of chronic neutropenia, with the most common type of chronic neutropenic disorder being idiopathic (~40,000), followed by cyclic (~5,000), and congenital (~3,000), and with the majority of affected people being female adults. Our research into the estimated individuals in the U.S. diagnosed with chronic neutropenia confirms significant unmet medical needs exist despite the availability and use of G-CSF and, if our analysis is correct, this suggests a potential minimal addressable market for mavorixafor of approximately one third of this population, or approximately 15,000 individuals in the U.S., plus meaningful potential market expansion opportunities.

In 2022, we also completed an electronic medical records study to better understand the risk of serious or severe infection in people with chronic neutropenia in the United States, analyzing the medical records of 44 healthcare organizations treating approximately 66 million patients. The analysis examined patients who had experienced at least two Serious Infections Events ("SIEs") following documentation of chronic neutropenia in each calendar year compared with those who did not have neutropenia. SIEs are defined as infections requiring hospitalization or intravenous antibiotics or that result in disability or death.

- The results of this analysis indicated that the incidence rate of SIEs per 100,000 person days was increased for all levels of chronic neutropenia: it was two times greater for patients with any chronic neutropenia (ANC less than 1,500 cells per microliter) and four times greater for patients with severe congenital neutropenia (ANC less than 500 cells per microliter).
- The risk of serious infection increased with the worsening of neutropenia.
- Approximately 25% of patients with chronic neutropenia had at least 2 SIEs in the latest calendar year examined, which was 2019.

People living with chronic neutropenia have few treatment options and may be treated with G-CSF, an injectable therapy approved in the United States for the treatment of severe, chronic neutropenia. G-CSF 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 chronic neutropenia 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 transplantation is often applied to severe neutropenia from bone marrow failure. Bone marrow transplants bring additional risks into the management of the disorders.

Clinical Development of Mavorixafor in Chronic Neutropenic Disorders

In 2022, we conducted a proof-of-concept Phase 1b open-label, multicenter study designed to assess the safety and tolerability of oral mavorixafor, with or without injectable G-CSF, in participants with chronic neutropenic disorders, including idiopathic, cyclic, and congenital neutropenia. Participants received a single dose of 400 mg oral mavorixafor to assess the magnitude of treatment response.

In September 2022, we announced positive results from the Phase 1b clinical trial:

- A total of 25 participants were enrolled in the study.
- 100% of study participants responded to treatment with a single dose of 400 mg of mavorixafor, alone or dosed concurrently with G-CSF:
    - Participants achieved a mean ANC increase at peak of >3,000 cells per microliter.
    - Consistent responses were seen across all of the chronic neutropenic disorders studied – idiopathic, cyclic, and congenital neutropenia.
- All neutropenic participants (n=14) reached normalized ANC levels (>1,500 cells per microliter):
    - When assessed as a monotherapy in participants with severe chronic neutropenia who were not being treated with G-CSF (n=6), a single dose of mavorixafor led to normalized ANC levels in all participants within 2 hours, with a mean ANC increase at peak of ~2,500 cells per microliter.
    - When assessed in participants with moderate or severe neutropenia, despite being treated with G-CSF (n=8), 100% reached normalized ANC levels, suggesting the potential of mavorixafor to both normalize the neutrophil counts in patients with partial response to G-CSF and also to potentially enable the reduction or elimination of G-CSF dosing.
- When assessed in participants with chronic neutropenia with normalized ANC counts on chronic G-CSF (n=11), all participants experienced a consistent and sustained increase in ANC, suggesting mavorixafor's potential to reduce or possibly eliminate G-CSF treatment in these patients.
- Mavorixafor was well tolerated in the study; all treatment-related adverse events were deemed to be low grade, consistent with previous clinical studies in WHIM syndrome, and no treatment-related serious adverse events were reported.

Following these positive results, an amendment to the Phase 1b clinical trial was initiated aiming to evaluate the use of daily oral mavorixafor with or without injectable G-CSF for up to 6 months in participants with chronic neutropenic disorders as a Phase 2 clinical trial. The ongoing Phase 2 trial is assessing the durability of ANC responses, the potential of mavorixafor to enable patients to taper down dosing with G-CSF, and to evaluate the tolerability of mavorixafor in combination with G-CSF in chronic use. The trial is now fully enrolled and we expect to report additional data in the second quarter of 2024.

Concurrent with this Phase 2 trial execution, we are advancing our plans to conduct a Phase 3 program of mavorixafor in people with certain chronic neutropenic disorders. The planned Phase 3 trial will be a global, randomized, double-blinded, placebo-controlled trial assessing the safety and efficacy of once-daily oral mavorixafor, with or without concomitant G-CSF, in people with idiopathic or congenital neutropenia. The 52-week trial is expected to enroll 150 participants aged 12 years and older with both an ANC less than 1,500 cells per microliter and 2 or more infections requiring intervention during the 12 months preceding the trial. The primary endpoint of the trial is a two-component endpoint that includes the annualized infection rate and ANC response in the mavorixafor-treated group versus the placebo group. Secondary endpoints are expected to include analysis of the severity and duration of infections, antibiotic use, fatigue, and quality of life parameters. The dosing of mavorixafor will be the same as in the 4WHIM Phase 3 clinical trial. The Company anticipates enrolling the first participants in this Phase 3 clinical trial in the second quarter of 2024.

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 to us, particularly through collaborative arrangements with large and established companies.

Our commercial opportunities 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 to buyers from a cost perspective.

We are aware of other companies that are developing injectable CXCR4 inhibitors. However, we are not aware of any companies with CXCR4 antagonist programs in development for the indications of WHIM syndrome or chronic neutropenia. With regard to chronic neutropenia, we are not aware of any companies who are developing an oral therapy to elevate neutrophils in the blood. Filgrastim injections (human G-CSF) and two biosimilars (Zarxio and Nivestym) are FDA approved to reduce the incidence and duration of after-effects of severe neutropenia (e.g. fever, infections, oropharyngeal ulcers) in symptomatic patients with severe neutropenia.

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 any of our product or product candidates.

We currently have a master services agreement, as amended from time to time, with Evotec A.G. (“Evotec”, previously known as Aptuit, Oxford), pursuant to which Evotec develops and manufactures the active pharmaceutical ingredient (“API”), mavorixafor. The term of the Evotec Agreement expires in February 2027 unless terminated by us and/or Evotec. Evotec is currently our sole supplier for mavorixafor drug substance. We are in the process of transitioning the Evotec Agreement to a commercial supply agreement to support our potential WHIM launch and subsequent commercial supply.

We also have a master services agreement in place with Catalent Inc. (“Catalent”), which is our sole manufacturer for the final capsule drug product formulation of mavorixafor. The term of the master services agreement with Catalent expires on September 10, 2024, and may be terminated by (1) us upon 30 days-notice to Catalent or (2) by either party following a material breach by the other party that remains uncured for 30 days. We are in the process of transitioning the master services agreement with Catalent to a commercial supply agreement to support our potential WHIM launch and subsequent commercial supply.

We obtain clinical, and potentially commercial, supplies from Evotec and Catalent pursuant to typical industry standard commercial and clinical supply agreements. We believe that both manufacturers have the capability and capacity to manufacture currently projected clinical trial supply and commercial volumes of mavorixafor.

Sales and Marketing

We are currently building a commercial infrastructure to support sales of mavorixafor should the FDA provide marketing approval for mavorixafor in the United States. We expect to manage sales, marketing and distribution through internal resources and third-party relationships. Upon obtaining approval of our NDA by the FDA, we plan to have a field sales force who will provide information about our approved drug product to health care providers who are known to or potentially could have WHIM patients under their care.

We have entered into agreements with a third-party logistics company (“3PL”) for the warehousing and distribution of our drug product. We have also entered into an agreement with a specialty pharmacy, who will purchase our labelled drug product and dispense such drug product to patients pursuant to prescriptions provided by health care providers. The specialty pharmacy will also serve as our point of contact for inbound health care provider and patient inquiries, prescription processing, insurance investigation and patient on-boarding.

We currently intend to file a marketing authorization application ("MAA") with the European Medicines Agency ("EMA") for the marketing of mavorixafor in the European Union. Prior to filing the MAA, we must complete a Pediatric Investigational Plan ("PIP"). We anticipate submitting an MAA with the EMA in the fourth quarter of 2024 or in early 2025.

License Agreement

License Agreement with Genzyme

In July 2014, we entered into a license agreement with Genzyme Corporation ("Genzyme"), a wholly owned subsidiary of Sanofi, 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. Genzyme has 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.

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.

During 2023, we achieved a regulatory milestone: the acceptance by the FDA of our first NDA, for which $5.0 million has been paid and expensed as research and development expense. As of December 31, 2023, we are obligated to pay Genzyme future milestone payments in the aggregate amount of up to $20 million, contingent upon our achievement of certain late-stage regulatory and sales milestones with respect to licensed products, and tiered royalties based on net sales of licensed products that we commercialize under the Genzyme agreement. The next potential regulatory milestone would be triggered upon the notification of regulatory approval of our NDA and would be in the amount of $7.0 million. The remaining regulatory milestones include (i) $3.0 million for the acceptance by the EMA of our first drug application and (ii) $5.0 million upon the notification by the EMA of regulatory approval of our first drug application. We must also make one-time sales milestone payments of $0.5 million, $1.5 million and $3.0 million upon achieving cumulative net sales of $50 million, $150 million and $300 million, respectively. Upon the first potential sale of our drug candidate in the U.S., we will incur a royalty on annual net sales at a rate of 6% up to $150 million, 10% on the portion of annual net sales between $150 million and $300 million, and 12% thereafter.

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. Sublicenses that we enter into under the Genzyme agreement obligate us 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 ("Georgetown") pursuant to which we obtained an exclusive, worldwide license to practice certain methods, and to make, have made, use, sell, offer for sale or 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 thousand, and we may be required to pay milestone payments of up to an aggregate of $800 thousand 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 that 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 ("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 BIDMC agreement.

Under the terms of the BIDMC agreement we paid a one-time-only, upfront fee of $20 thousand 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 BIDMC agreement with respect to the country or countries in which such material breach has occurred. We may terminate the BIDMC agreement at any time upon at least 90 days' written notice.

License Agreement with Dana Farber Cancer Institute

In November 2020, we entered into a license agreement with Dana Farber Cancer Institute ("DFCI") pursuant to which we obtained a non-exclusive, worldwide license to use, make, have made, develop, market, import, distribute, sell or have sold licensed products and certain processes covered by licensed patent rights owned by DFCI. The rights licensed to us are for the therapeutic use of our CXCR4 antagonists for the treatment of Waldenström's in combination with BTK inhibitors, including ibrutinib. We have the right to grant sublicenses of the licensed rights to third parties to the extent consistent with the terms of the DFCI agreement.

Under the terms of the DFCI agreement we paid a one-time, upfront fee of $25 thousand and we are responsible for the reimbursement of certain future patent prosecution costs and the payment of an annual maintenance fee. We are obligated to pay DFCI milestone payments in the aggregate amount of up to approximately $32 million, contingent upon our achievement of certain regulatory and sales milestones with respect to licensed products, and a flat royalty based on net sales of licensed products

that we commercialize under the DFCI agreement.

The term of the DFCI agreement will continue until the expiration of the last valid claim within the patent rights covering the licensed product. DFCI may terminate the DFCI agreement 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 cease to carry on our business with respect to the licensed products or process, (iii) the insurance coverage that we are obligated to maintain under the DCFI agreement is terminated and we fail to obtain replacement insurance within a certain period of time following notice to DFCI, (iv) we fail to comply with certain diligence obligations and cure any such default within 60 days after receipt of written notice, (v) we have granted a sublicense without notifying DFCI or on terms inconsistent with the terms required of sublicenses under the DCFI agreement, (vi) an officer of our company, an affiliate or sublicensee is convicted of a felony relating to the manufacture, use, sale or importation of a licensed product, (vii) we or any of our affiliates, sublicensees or sublicensees' affiliates initiate a patent challenge of the patents licensed under the DFCI agreement or assists others in doing so or (viii) we declare insolvency or bankruptcy. In addition, if we are in material breach of any material obligations under the DFCI agreement and fail to remedy such breach within 90 days after receipt of notice, DFCI may terminate the DFCI agreement or terminate any licenses granted under the agreement. We may terminate the DFCI 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. ("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 are expected to 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 entered into a separate agreement in April 2020 whereby we will provide Abbisko with a clinical supply and, if the product is commercialized in the territory licensed by Abbisko, we intend to enter into a commercial supply of the licensed compound.

Pursuant to the agreement with Abbisko, upon the closing of a qualified financing of Abbisko, as defined in the agreement, which occurred in March 2020, Abbisko made a one-time, non-refundable, non-creditable financial milestone payment of $3 million to us. We are also eligible to receive potential development, regulatory and commercial milestone payments of up to $214.0 million, which will vary based on the ultimate sales, if any, of the approved 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, 2023, we owned or exclusively licensed 20 issued U.S. patents, five pending U.S. non-provisional patent applications, three pending U.S. provisional patent applications, and approximately 160 PCT and foreign patents and patent applications including in the following foreign jurisdictions: Austria, Australia, Belgium, Brazil, Canada, China, Denmark, European Patent Office, Finland, France, Germany, Great Britain, Hong Kong, India, Ireland, Israel, Italy, Japan, Lichtenstein, Mexico, Netherlands, New Zealand, Norway, Poland, Portugal, Singapore, South Africa, South Korea, Spain, Sweden and Switzerland.

Patents issued from the in-licensed portfolio are exclusively licensed to us under the terms of the Genzyme Agreement and are expected to expire between 2024 and 2027. Additionally, we have exclusively licensed from Genzyme, BIDMC and Georgetown University their interest in certain co-owned patents. Patents issued from our co-owned portfolio, if all maintenance fees are paid,

are expected to expire in 2036, not including any Patent Term Adjustment (PTA), Patent Term Extension (PTE), or other extensions of term that may be available.

With respect to our lead product mavorixafor, as of December 31, 2023, we owned or exclusively licensed five issued U.S. patents and one pending U.S. non-provisional patent application that relate to mavorixafor composition of matter; one issued U.S. patent, one pending PCT application, and three pending foreign priority patent applications that relate to methods of manufacturing mavorixafor, including certain key intermediate molecules; two issued U.S. patents and one pending U.S. non-provisional patent application that relate to the use of mavorixafor for treatment of patients with WHIM Syndrome; one pending U.S. non-provisional patent application, one pending PCT application, and one pending U.S. provisional patent application that relate to the use of mavorixafor for treatment of patients with conditions involving chronic neutropenia; and three U.S. issued patents, one pending U.S. non-provisional patent application, and three pending PCT applications that relate to uses of mavorixafor in other fields, including oncology. The issued U.S. patents covering aspects of mavorixafor and its use, if all maintenance fees are paid, and pending applications, if granted and all maintenance fees paid, are expected to expire between 2024 and 2044, not including any Patent Term Adjustment (PTA), Patent Term Extension (PTE), or other extensions of term that may be available. With respect to foreign patent rights to mavorixafor, as of December 31, 2023, we had approximately 110 pending PCT and foreign patents and patent applications.

With respect to developmental compounds, including preclinical candidates for our X4P-002 and X4P-003 programs, as of December 31, 2023, we had nine issued U.S. patents, one pending U.S. non-provisional patent application, two pending U.S. provisional patent applications and two pending PCT patent applications. These issued patents, if all maintenance fees are paid, and pending applications, if granted and all maintenance fees paid, are expected to expire between 2024 and 2043, not including any Patent Term Adjustment (PTA), Patent Term Extension (PTE), or other extensions of term that may be available. With respect to foreign patent rights to our developmental compounds, including preclinical candidates for our X4P-002 and XP-003 programs, we have approximately 50 pending PCT and foreign patents and patent applications.

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, (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 (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 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 and well-controlled investigations demonstrating that the drug is safe for use under the conditions prescribed, recommended or suggested in the proposed labeling and providing 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.

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 certain nonclinical tests must comply with federal requirements, including, as applicable, the FDA's good laboratory practices regulations and the U.S. Department of Agriculture'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 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 in compliance with applicable federal regulations, including good clinical practices, which are meant to protect the rights and safety of study subjects and ensure the integrity of the data generated in the clinical trials and 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 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 applicable requirements or presents an unacceptable risk to the clinical trial patients. The trial protocol and informed consent information for patients must also be submitted to an institutional review board ("IRB") at each site where a trial will be conducted. 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 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 drug 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 safety and efficacy of the drug. The FDA may, however, determine that a drug is safe and effective based on one clinical trial plus confirmatory evidence. 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 relevant preclinical, clinical, and other testing and data and information relating to the product's pharmacology, chemistry, manufacture, and controls. In addition, the submission of NDAs is generally subject to a substantial application fee, although there are 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 performance goals established pursuant to the Prescription Drug User Fee Act the FDA aims to complete review of 90% of standard (non-priority) NDAs within 10 months of filing and within six months of filing for priority NDAs.

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 on questions presented by the FDA, which may include questions related 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 to assess compliance with cGMP.

After the FDA evaluates the NDA and has conducted applicable inspections, it issues either an approval letter or a complete response letter. A complete response letter generally outlines the deficiencies in the submission and actions the sponsor may take, such as additional testing, or information, in order for the FDA to reconsider the application. If, and when, those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the NDA, the FDA will issue an approval letter.

An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. An approval letter may also include post-marketing requirements and commitments, such as the conduct of additional clinical trials or CMC studies. As a condition of NDA approval, the FDA may require a risk evaluation and mitigation strategy ("REMS") to 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 ("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.

The Hatch-Waxman Act

Orange Book Listing

When seeking NDA approval, 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 ("Orange Book"). Drugs listed in the Orange Book can, in turn, be referenced by potential competitors in support of approval of an abbreviated new drug application ("ANDA") or an NDA submitted under section 505(b)(2) of the FD&C Act ("505(b)(2) NDA"). 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 to be bioequivalent to the listed drug. Other than bioequivalence testing, ANDA applicants are not required to conduct, or submit results of, pre-clinical or clinical tests to demonstrates the safety or effectiveness of their drug

product. Drugs approved in this way generally 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. A 505(b)(2) NDA provides for the marketing of a drug for which one or more investigations supporting its approval were not conducted by or for the applicant or for which the applicant had not obtained a right of reference. In some instances, a 505(b)(2) NDA applicant may rely on the FDA's findings of safety and effectiveness for a previously approved drug.

An ANDA applicant, or a 505(b)(2) NDA applicant that is relying on FDA's finding of safety and effectiveness for a previously approved drug, 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 applicant may also elect to submit a section viii statement, certifying that the proposed product labeling does not contain (or carves out) any language related to the listed the 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 an ANDA or 505(b)(2) NDA will not infringe the already approved product's listed patents, or that such patents are invalid, is called a Paragraph IV certification. If the 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 application 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 notice automatically prevents the FDA from approving the ANDA or 505(b)(2) NDA 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 applicant.

The ANDA or 505(b)(2) NDA 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 ("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) NDA application seeking approval of a drug that references the NCE drug. Certain changes to an approved drug that are supported by clinical studies that are essential to the approval of such changes, such as the addition of a new indication, are associated with a three-year period of exclusivity during which the FDA cannot approve an ANDA or 505(b)(2) NDA application that includes the change.

An ANDA or 505(b)(2) NDA 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.

Five-year and three-year exclusivities do not preclude FDA approval of another 505(b)(1) NDA application for 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 generally is calculated based on half of the drug's testing phase—the time between IND clearance and NDA submission—and all of the review phase—the time between NDA submission and approval up to a maximum of five years. The extension period 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.

Postmarket Requirements

Once an NDA is approved, the manufacturer and the product will be subject to continuing regulation by the FDA, including, among other things, monitoring and recordkeeping activities, annual report requirements, reporting of adverse experiences, and complying with promotion and advertising requirements. The FDA closely regulates the post-approval marketing and promotion of drugs.

Drugs may be marketed only for the approved indications and in a manner that is consistent with their approved labeling. Although physicians may prescribe legally available products for off-label uses, manufacturers may not market or promote such uses. Prescription drug promotional materials must be submitted to the FDA at the time of their first use.

Changes to certain 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 similar procedures in reviewing and approving NDA supplements as it does for original NDAs.

Adverse event reporting and submission of annual safety reports is also required following FDA approval of an NDA. New safety information that emerges after NDA approval may require changes to a drug's approved labeling, including the addition of new warnings and precautions or contraindications, and could require the implementation of other risk management measures, including a REMS or the conduct of post-marketing studies to assess the newly discovered safety issue. Product approvals also may be withdrawn if problems occur following initial marketing.

In addition, quality control, drug manufacture, packaging, and labeling procedures must continue to conform to cGMP 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 cGMP. Manufacturers are also subject to tracking and tracing requirements. Accordingly, manufacturers must continue to expend time, money and effort in the areas of production and quality control to maintain compliance with cGMP. 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 Information

The Best Pharmaceuticals for Children Act ("BPCA") provides NDA holders a six-month period of exclusivity attached to any patent or regulatory exclusivity listed in the Orange Book 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, completion of the studies in accordance with the written request and submission of reports from the requested studies to the FDA.

In addition, under the Pediatric Research Equity Act ("PREA"), certain NDAs or NDA supplements 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 may request a deferral or waiver 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.

Orphan Drug and Rare Pediatric Disease Designation

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 designated 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 drug 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 drug, for that disease. 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. The FDA must also publish a summary of its clinical superiority findings upon granting approval and orphan drug exclusivity to a subsequent product 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 fee.

The FDA grants Rare Pediatric Disease designation for serious and life-threatening diseases that primarily affect children ages 18 years or younger and fewer than 200,000 individuals in the United States. A priority review voucher may be issued upon approval of an NDA for therapies developed to treat such rare pediatric diseases. Priority review vouchers may be redeemed to obtain priority review for any subsequent marketing application or be sold or transferred.

Expedited Development and Review Programs

The FDA has a Fast Track program that is intended to expedite or facilitate the process for development and review of new drugs that meet certain criteria. Specifically, new drugs are eligible for Fast Track designation if they are intended to treat a serious or life-threatening condition and preclinical or clinical data demonstrate the potential to address unmet medical needs for the condition. Fast Track designation applies to both the product and the specific indication for which it is being studied. Products that receive Fast Track designation may also be eligible for other types of FDA programs intended to expedite development and review, such as priority review and accelerated approval.

Any product is eligible for priority review if it treats a serious or life-threatening condition and, if approved, would provide a significant improvement in safety and effectiveness compared to available therapies. The FDA will attempt to direct additional resources to the evaluation of an application for a new drug designated for priority review to facilitate the review.

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 afterward, but ideally before an end-of-Phase-2 meeting. 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.

Rest of World Government Regulation

In addition to laws and 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, manufacturing and any commercial sales and distribution of our products, if approved.

As in the United States, we must obtain the requisite authorization or approvals from regulatory authorities in non-U.S. countries prior to the commencement of clinical trials or marketing of the product in those countries. The requirements of regulatory authorities outside the United States are in many respects similar to those we are subject to in the United States, but in some instances the legal and regulatory requirements outside the United States may differ from or be more stringent than what we must comply with in the United States.

European Union Drug Development

In the European Union ("EU"), our future products may also be subject to extensive regulatory requirements. As in the United States, medicinal products can be marketed only if a marketing authorization from the competent regulatory agencies has been obtained.

Similar to the United States, the various phases of preclinical and clinical research in the EU are subject to significant regulatory controls. In April 2014, the EU adopted the new Clinical Trials Regulation (EU) No 536/2014, which replaced the Clinical Trials Directive 2001/20/EC on January 31, 2022. The transitory provisions of the new Regulation provide that, by January 31, 2025, all ongoing clinical trials must have transitioned to the new Regulation.

The new Regulation overhauled the system of approvals for clinical trials in the EU. Specifically, it is directly applicable in all Member States (meaning that no national implementing legislation in each Member State is required), and aims at simplifying and streamlining the approval of clinical trials in the EU. The main characteristics of the new Regulation include: a streamlined application procedure via a single-entry point through the Clinical Trials Information System ("CTIS"); a single set of documents to be prepared and submitted for the application as well as simplified reporting procedures for clinical trial sponsors; and a harmonized procedure for the assessment of applications for clinical trials, which is divided in two parts (Part I contains scientific and medicinal product documentation and Part II contains the national and patient-level documentation). Part I is assessed by a coordinated review by the competent authorities of all EU Member States in which an application for authorization of a clinical trial has been submitted (Member States concerned) of a draft report prepared by a Reference Member State. Part II is assessed separately by each Member State concerned. Strict deadlines have also been established for the assessment of clinical trial applications.

European Union Drug Review and Approval

In the EU, medicinal products can only be commercialized after obtaining a marketing authorization ("MA").

The centralized MA is issued by the European Commission through the centralized procedure, based on the opinion of the Committee for Medicinal Products for Human Use ("CHMP"), of the EMA, and is valid throughout the entire territory of the EU and the additional Member States of the European Economic Area (Iceland, Liechtenstein and Norway) ("EEA"). The centralized procedure is mandatory for certain types of products, including products produced by biotechnological processes, products designated as orphan medicinal products, advanced-therapy medicinal products (gene-therapy, somatic cell-therapy, or tissue-engineered medicines) and medicinal products containing a new active substance indicated for the treatment of HIV, AIDS, cancer, neurodegenerative disorders, diabetes, auto-immune and other immune dysfunctions or viral diseases. The centralized procedure is optional for products containing a new active substance not yet authorized in the EU, or for products that constitute a significant therapeutic, scientific, or technical innovation or which are in the interest of public health in the EU.

Under the centralized procedure, the EMA's CHMP is responsible for conducting the initial assessment of a product and for several post-authorization and maintenance activities, such as the assessment of modifications or extensions to an existing MA. The maximum timeframe for the evaluation of an MA 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. Clock stops may extend the timeframe of evaluation of an MA application considerably beyond 210 days. Where the CHMP gives a positive opinion, it provides the opinion together with supporting documentation to the European Commission, who makes the final decision to grant an MA, which is issued within 67 days of receipt of the EMA's recommendation. Accelerated assessment might be granted by the CHMP in exceptional cases, when a medicinal product is expected to be of major public health interest, particularly from the point of view of therapeutic innovation. If the CHMP accepts such request, the time limit of 210 days will be reduced to 150 days, excluding clock stops, but it is possible that the CHMP may revert to the standard time limit for the centralized procedure if it determines that the application is no longer appropriate to conduct an accelerated assessment.

European Union New Chemical Entity Exclusivity

In the EU, innovative medicinal products approved on the basis of a complete and independent data package qualify for eight years of data exclusivity upon the grant of an MA and an additional two years of market exclusivity. The data exclusivity, if granted, prevents generic or biosimilar applicants from referencing the innovator's preclinical and clinical trial data contained in the dossier of the reference product when applying for a generic or biosimilar MA in the EU, during a period of eight years from the date on which the reference product was first authorized in the EU. During the additional two-year period of market exclusivity, a generic or biosimilar MA application can be submitted and authorized, and the innovator's data may be referenced,

but no generic or biosimilar product can be placed on the EU market until the expiration of the market exclusivity. The overall ten-year period will be extended to a maximum of eleven years if, during the first eight years of those ten years, the MA holder obtains an MA for one or more new therapeutic indications which, during the scientific evaluation prior to their MA, are determined to bring a significant clinical benefit in comparison with currently approved therapies. There is no guarantee that a product will be considered by the EMA to be an innovative medicinal product, and products may not qualify for data exclusivity. Even if a product is considered to be an innovative medicinal product so that the innovator gains the prescribed period of data exclusivity, another company could nevertheless also market another version of the product if such company obtained an MA based on an MA application with a complete and independent data package of pharmaceutical tests, preclinical tests and clinical trials.

European Union Orphan Designation and Exclusivity

In the EU, the European Commission grants orphan designation in respect of a product, after receiving the opinion of the EMA's Committee for Orphan Medicinal Products, if its sponsor can establish that: (1) the product is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition; (2) either (i) such condition affects no more than five in 10,000 persons in the EU when the application is made, or (ii) it is unlikely that the product, without the benefits derived from orphan status, would generate sufficient return in the EU to justify the necessary investment in its development; and (3) there exists no satisfactory method of diagnosis, prevention or treatment of such condition authorized for marketing in the EU, or, if such a method exists, the product would be a significant benefit to those affected by that condition.

In the EU, orphan designation entitles a party to financial incentives such as reduction of fees or fee waivers and 10 years of market exclusivity is granted following the grant of an MA. During this market exclusivity period, neither the EMA nor the European Commission nor any of the competent authorities in the EU Members States can accept an application or grant an MA for a "similar medicinal product." A "similar medicinal product" is defined as a medicinal product containing a similar active substance or substances as contained in an authorized orphan medicinal product, and which is intended for the same therapeutic indication. This period may be reduced to six years if, at the end of the fifth year, it is established that the orphan designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. An MA may be granted to a similar medicinal product to an authorized orphan product in very select cases, such as if: (i) it is established that a similar medicinal product is safer, more effective or otherwise clinically superior to the authorized product; (ii) the MA holder for the authorized orphan product consents to the authorization of the similar medicinal product; or (iii) the MA holder for the authorized orphan product cannot supply enough orphan medicinal product. Orphan designation must be requested before submitting an application for an MA. Orphan designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

European Union Regulatory Requirements After a Marketing Authorization has been Obtained

If authorization for a medicinal product in the EU is obtained, the holder of the MA is required to comply with a range of requirements applicable to the manufacturing, marketing, promotion, and sale of medicinal products. These include:

- Compliance with the EU's stringent pharmacovigilance or safety reporting rules must be ensured. These rules can impose post-authorization studies and additional monitoring obligations.
- The manufacturing of authorized medicinal products, for which a separate manufacturer's license is mandatory, must also be conducted in strict compliance with the applicable EU laws, regulations and guidance, including Directive 2001/83/EC, Directive 2017/1572, Regulation (EC) No 726/2004 and the European Commission Guidelines for Good Manufacturing Practice ("EU cGMP"). These requirements include compliance with EU cGMP standards when manufacturing medicinal products and active pharmaceutical ingredients, including the manufacture of active pharmaceutical ingredients outside of the EU with the intention to import the active pharmaceutical ingredients into the EU.
- Much like the Anti-Kickback Statute prohibition in the United States, the provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order, or use of medicinal products is also prohibited in the EU. The provision of benefits or advantages to physicians may be governed by the national anti-bribery laws of EU Member States, and the Bribery Act 2010 in the UK. Infringement of these laws could result in substantial fines and imprisonment. EU Directive 2001/83/EC, which is the EU Directive governing medicinal products for human use, further provides that, where medicinal products are being promoted to persons qualified to prescribe or supply them, no gifts, pecuniary advantages, or benefits in kind may be supplied, offered or

promised to such persons unless they are inexpensive and relevant to the practice of medicine or pharmacy. This provision has been transposed into the Human Medicines Regulations 2012 and so remains applicable in the UK despite its departure from the EU.

Payments made to physicians in certain EU Member States must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization as well as the regulatory authorities of the individual EU Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct, applicable in the EU Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

The aforementioned EU rules are generally applicable in the EEA.

Pharmaceutical Coverage, Pricing and 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 payors, such as government health programs, and commercial insurance and managed health care organizations. These third-party payors are increasingly challenging the prices charged for medical products and services.

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.

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 payors do not consider our products to be cost-effective compared to other available therapies, they may not cover our products after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow us to sell our products on a profitable basis.

The Medicare Prescription Drug, Improvement, and Modernization Act of 2003 ("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 ("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 payors 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 payors.

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.

Healthcare Reform

Payors, whether domestic, foreign, governmental or private, are developing increasingly sophisticated methods of controlling healthcare costs. In both the U.S. and certain foreign jurisdictions, there have been a number of legislative and regulatory changes to the health care system that could impact our ability to sell our products profitably. For example, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education 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.

Since its enactment, there have been a number of judicial, administrative, executive, and legislative challenges to certain aspects of the ACA. For example, President Biden has issued multiple executive orders that have sought to reduce prescription drug costs. Further, on June 17, 2021, the U.S. Supreme Court dismissed a judicial challenge on procedural grounds that argued the ACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress. In addition, the Inflation Reduction Act of 2022 ("IRA"), among other things, extends enhanced subsidies for individuals purchasing health insurance coverage in ACA marketplaces through plan year 2025. The IRA also eliminates the "donut hole" under the Medicare Part D program beginning in 2025 by capping the beneficiary maximum out-of-pocket cost at $2,000 per year and creating a new manufacturer discount program. It is possible that the ACA will be subject to judicial or Congressional challenges in the future. It is unclear how any such challenges and the healthcare reform measures of the Biden administration will impact the ACA.

Also, there has been heightened governmental scrutiny recently over pharmaceutical pricing practices in light of the rising cost of prescription drugs and biologics. Such scrutiny has resulted in several Presidential executive orders, Congressional hearings and proposed and enacted federal and state legislation 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 products. Congress has also passed additional reform measures. This includes the IRA, which, (i) directs HHS to negotiate the price of certain high-expenditure, single-source drugs and biologics covered under Medicare, and subject drug manufacturers to civil monetary penalties and a potential excise tax by offering a price that is not equal to or less than the negotiated "maximum fair price" for such drugs and biologics under the law, and (ii) requires drug manufacturers to pay drug rebates with respect to certain drugs and biologics covered under Medicare Part B or Medicare Part D to the extent that drug prices increase faster than inflation. Under the IRA, orphan drugs are exempted from the Medicare drug price negotiation program, but only if they have one orphan designation and for which the only approved indication(s) is for that disease or condition. The IRA permits HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years. These provisions will take effect progressively starting in fiscal year 2023, although the implementation of the IRA is currently subject to ongoing litigation that challenges the constitutionality of the IRA's Medicare drug price negotiation program. It is currently unclear how the IRA will be implemented but is likely to have a significant impact on the pharmaceutical industry. Further, the Biden administration released an additional executive order on October 14, 2022, directing HHS to submit a report on how the Center for Medicare and Medicaid Innovation can be further leveraged to test new models for lowering drug costs for Medicare and Medicaid beneficiaries. It is unclear whether this executive order or similar policy initiatives will be implemented in the future.

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.

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 payors that are false or fraudulent;
- the federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), which created additional federal criminal statutes that prohibit, among other things, 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, (currently defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain other healthcare professionals (such as physician assistants and nurse practitioners) and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members;
- HIPAA, as amended by the Health Information Technology and Clinical Health Act ("HITECH"), and its implementing regulations, which imposes certain requirements on "covered entities," including certain healthcare providers, health plans, and healthcare clearinghouses, as well as their respective "business associates" that create, receive, maintain or transmit individually identifiable health information for or on behalf of a covered entity, and their covered subcontractors, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- Federal drug price reporting laws, which require manufacturers to calculate and report complex pricing metrics to government programs, where such reported prices may be used in the calculation of reimbursement and/or discounts on approved products; 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 payor, 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.

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 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.

Human Capital Policies and Procedures

As of December 31, 2023, we had 93 full-time employees. Of these employees, 58 were engaged in research and development and 35 were engaged in selling, general and administrative functions. All of our employees are located in the United States or 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.

Human capital is critical to our success. Our overarching human capital resource strategy is to recruit, hire, incentivize and retain employees consistent with our stage of operations and strategic objectives. We believe we offer our employees compensation that is competitive and consistent with the markets in which we operate, namely the Greater Boston and the Vienna, Austria metropolitan areas. We supplement base cash employee compensation with awards of stock options and/or restricted stock units under our equity incentive plans. We review employee performance annually and our Compensation Committee approves associated merit increases and annual incentive bonus payments during the first quarter of the year annually. When needed, we augment our employee base with outside consultants who specialize in various fields.

Corporate Information and Trademarks

We were incorporated under the laws of the State of Delaware in 2010 under the name Arsanis Inc. Following the Merger with X4 Therapeutics Inc. (formerly X4 Pharmaceuticals Inc.) on March 13, 2019, we changed our name to X4 Pharmaceuticals, Inc. Our principal executive offices are located at 61 North Beacon Street, 4th Floor, Boston, Massachusetts 02134 and our telephone number is (857) 529-8300.

We view our operations and measure our business as one reportable segment. All of the Company's tangible assets are held in the United States. Refer to Note 2, Summary of Significant Accounting Policies, to our financial statements appearing elsewhere in this Annual Report on Form 10-K for additional information.

We own or have rights to various trademarks, service marks and trade names that we use in connection with the operation of our business. This Annual Report on Form 10-K may also contain trademarks, service marks and trade names of third parties, which are the property of their respective owners. Our use or display of third parties' trademarks, service marks, trade names or products in this Annual Report on Form 10-K is not intended to, and does not imply a relationship with, or endorsement or sponsorship by us. Solely for convenience, the trademarks, service marks and trade names referred to in this Annual Report on Form 10-K may appear without the ®, ™ or SM symbols, but the omission of such references is not intended to indicate, in any way, that we will not assert, to the fullest extent under applicable law, our rights or the right of the applicable owner of these trademarks, service marks and trade names.

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. We do not incorporate the information on or accessible through our website into this Annual Report on Form 10-K, and you should not consider any information on, or that can be accessed through, our website to be part of this Annual Report on Form 10-K. We have included our website address in this Annual Report on Form 10-K solely as an inactive textual reference.

ITEM 1A. RISK FACTORS

An investment in our securities involves a high degree of risk. You should carefully consider the following information about these risks, together with the other information appearing elsewhere in this Annual Report, including our audited consolidated financial statements and related notes hereto, 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, or cause our actual results to differ materially from those contained in forward-looking statements we have made in this report and those we may make from time to time. In these circumstances, the market price of our common stock could decline, and you may lose all or part of your investment. We cannot assure you that any of the events discussed below will not occur.

Summary of Selected Risks Associated with Our Business

Our business faces significant risks and uncertainties. If any of the following risks are realized, our business, financial condition and results of operations could be materially and adversely affected. You should carefully review and consider the full discussion of our risk factors in the section titled "Risk Factors" in Part I, Item 1A of this Annual Report. Some of the more significant risks include the following:

- We have incurred significant losses and have not generated revenue from product sales since our inception. We expect to continue to incur losses for the foreseeable future, and we may never achieve or maintain profitability.
- Our liquidity position raises substantial doubt about our ability to continue as a going concern and 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.
- 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.
- We depend almost entirely on the success of our lead product candidate, mavorixafor, which we are developing for the potential treatment of chronic neutropenic disorders, including WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) syndrome and, contingent on a potential strategic partnerships, for the treatment of Waldenström's. We cannot be certain that we will be able to obtain regulatory approval for, or successfully commercialize, mavorixafor or any other product candidate.
- 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.
- We depend on license agreements with Genzyme, Beth Israel Deaconess Medical Center, Georgetown University and Dana-Farber Cancer Institute 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.
- The results of clinical trials may not support our product candidate claims.
- 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.
- If the commercial opportunity for mavorixafor in WHIM syndrome and other chronic neutropenic disorders 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.
- Interim top-line and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

- 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. Additionally, 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.

- The FDA and other regulatory agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. If we are found or alleged to have improperly promoted off-label uses, we may become subject to significant liability.

- A breakthrough therapy designation or Fast Track designation by the FDA for our product candidates may not lead to a faster development or regulatory review or approval process, and neither of these designations increases the likelihood that our product candidates will receive marketing approval.

- 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.

- Even if we obtain and maintain approval for our product candidates from the FDA, we may never obtain approval for our product candidates outside of the United States, which would limit our market opportunities and could harm our business.

- 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.

- We have no 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 ("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 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.

- Disruptions in our supply chain could delay the commercial launch 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 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.

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

- 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 future success depends on our ability to retain executives and to attract, retain and motivate key personnel in a competitive environment for skilled biotechnology personnel.

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

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

- Our business could be adversely affected by economic downturns, inflation, increases in interest rates, natural disasters, public health crises, political crises, geopolitical events, such as the wars in Ukraine and Gaza, or other macroeconomic conditions, which have in the past and may in the future negatively impact our business and financial performance.

- Our stock price has been and is likely to continue to be volatile and fluctuate substantially.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred significant losses and have not generated revenue from product sales since our inception. We expect to continue to incur losses for the foreseeable future and we may never achieve or maintain profitability.

We are a late clinical-stage biopharmaceutical company. Investment in biopharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate effect or an acceptable safety profile, gain regulatory approval or become commercially viable. Since inception, we have incurred significant operating losses. Our net losses were $101.2 million, $93.9 million and $88.7 million for the years ended December 31, 2023, 2022 and 2021 respectively, and we had an accumulated deficit of $477.9 million as of December 31, 2023. 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. We have no products approved for commercial sale and have not generated any revenue from product sales to date, and we may never generate product revenue or achieve profitability.

We expect to continue to incur significant expenses and increasing operating losses for at least the next several 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.

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

- outcomes and timing of regulatory reviews, approvals and other actions;
- 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;
- 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 2 clinical trial of mavorixafor for the treatment of chronic neutropenic disorders;
- our ability to raise sufficient funds to support the development and potential commercialization of our product candidates;
- 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;
- the number and characteristics of product candidates and programs that we pursue;
- hire additional clinical, regulatory and scientific personnel; and

- incur additional legal, accounting and other expenses associated with operating as a public company.

Based on our current plans, 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.

We may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors that may alter or delay our plans. For example, we experienced delays in clinical trial site activation and slower patient enrollment in some of our clinical trials as a result of the recent COVID-19 pandemic, which delayed our expectations regarding our ability to report data from those trials. Assuming that we complete the development of and obtain marketing approval for any of our product candidates, including mavorixafor, which has a PDUFA target action date of April 30, 2024, 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.

Our liquidity position raises substantial doubt about our ability to continue as a going concern and 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.

We may be forced to delay or reduce the scope of our development programs and/or limit or cease our operations if we are unable to obtain additional funding to support our current operating plan. We have identified conditions and events that raise substantial doubt about our ability to continue as a going concern.

Our operations have consumed a large amount of cash since inception. To date, we have funded our operations 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. 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 U.S. 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.

As of December 31, 2023, we have cash and cash equivalents of $99.2 million and short-term marketable securities of $15.0 million. We will require additional capital to sustain our operations, and to carry out our business plans, which may include raising funds through public or private equity or debt financings, third-party funding, marketing and distribution arrangements, as well as other collaborations, strategic alliances and licensing arrangements, or any combination of these approaches. While we have successfully raised capital in the past, our ability to raise capital in future periods is not assured. We will also require additional capital to satisfy the covenant under our existing debt facility with Hercules Capital, Inc. and certain affiliated entities ("Hercules") that requires that we maintain a minimum level of cash of $20.0 million through January 2025 and thereafter, subject to reductions upon the Company's achievement of certain operational milestones. Based on our current cash flow projections, excluding any gross profit related to the potential sale of our drug, should the FDA approve our current NDA, and excluding the potential sale of any priority review voucher that might be received upon approval of the Company's NDA noted above, and with no additional borrowings under our existing debt facility or other sources of external financing, we would fail to maintain the minimum cash required to satisfy this covenant as soon as the first quarter of 2025. In such event, Hercules could require the repayment of all outstanding debt. Based on the foregoing, we have concluded that substantial doubt exists about our ability to continue as a going concern for a period of at least 12 months from the date of issuance of the financial statements appearing elsewhere in this Annual Report. Our financial statements have been prepared assuming that we will continue as a going concern, which contemplates the realization of assets and the settlement of liabilities and commitments in the normal course of business. The financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might result from the outcome of the uncertainty described above. See also the risk factor titled "*Our term loan contains restrictions that limit our flexibility in operating our business*" below.

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 us, 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. In addition, when we need to secure additional financing, such additional fundraising efforts may divert our management from our day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. Any of these events could significantly harm our business, financial condition and prospects, and our stockholders could lose all or part of their investment in our company.

We also could be required to:

- seek new or additional 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 outcome, timing and cost of regulatory reviews, approvals or other actions to meet regulatory requirements established by the U.S. Food and Drug Administration ("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 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;
- 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 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 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;
- market acceptance of our product candidates, to the extent any are approved for commercial sale;
- the effect of competing technological and market developments;
- the costs to operate as a public company; and

- business interruptions resulting from pandemics and public health emergencies, geopolitical actions, including war and terrorism or natural disasters including earthquakes, typhoons, floods and fires.

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 public or private equity or debt financings, third-party funding, marketing and distribution arrangements, as well as other collaborations, strategic alliances and licensing arrangements, or any combination of these approaches. Other than our common stock purchase agreement with Lincoln Park Capital Fund LLC ("Lincoln Park"), pursuant to which Lincoln Park is obligated, subject to certain limitations and conditions, to purchase up to a remaining $47.0 million in the aggregate of shares of our common stock, 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 additional assets such as intellectual property. For example, our debt facility with Hercules contains a minimum cash financial covenant that we project we would be in violation of in the first quarter of 2025 based on our current cash flow projections, assuming we do not raise additional funding and our drug candidate is not approved by the FDA. If we default on such indebtedness, with Hercules or a future lender, we could be required to pledge additional assets, or the lenders could enforce remedies on the current collateral.

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 revenues from any product sales since inception and may never become profitable.
To date, we have not generated revenues from any product sales. Our ability to generate revenue and become profitable depends upon our ability to successfully obtain marketing approval and commercialize our product candidates, including mavorixafor, 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 any of our current or future 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 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.

Changes in estimates regarding fair value of intangible assets may result in an adverse impact on our results of operations. We test goodwill for impairment annually or more frequently if changes in circumstances or the occurrence of events suggest impairment exists. Any significant change in market conditions, including a sustained decline in our stock price, that indicate a reduction in carrying value may give rise to impairment in the period that the change becomes known. For example, as of December 31, 2021, our market capitalization, measured as the price of our common stock multiplied by shares of common stock outstanding, declined to below the value of our net assets, including goodwill. As a result of the sustained decline in the market price of our common stock, the fair value of our single reporting unit, measured based on our market capitalization as of December 31, 2021, was lower than its carrying value and we concluded that goodwill was impaired. Accordingly, we recorded an impairment charge of $9.8 million to reduce the carrying amount of goodwill to $17.4 million as of December 31, 2021. While we determined that goodwill was not impaired based on its quantitative test as of December 31, 2023, future declines in the market value of our common stock may result in additional impairment charges being recorded.

Risks Related to Development of Our Product Candidates

We depend almost entirely on the success of our lead product candidate, mavorixafor, which we are developing for the potential treatment of WHIM syndrome and other chronic neutropenic disorders, and, contingent on a potential strategic partnership, for the treatment of Waldenström's. 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 products for sale and may never be able to develop marketable drug products. 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 also may not be able to finalize the design or formulation or 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 may develop mavorixafor, and potentially future product candidates, in combination with other therapies, which could expose us to additional risks.

We may develop mavorixafor, and may develop future product candidates, in combination with one or more currently approved 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 diseases, and we would be subject to similar risks if we develop any of our product candidates for use in combination with other drugs. 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 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, 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 other 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.

We are not permitted to market mavorixafor or any other product candidate in the United States until we receive approval of an New Drug Application ("NDA") from the FDA, or in any foreign countries until we receive the requisite approval from such countries or jurisdictions, such as approval of the marketing authorization application in the European Union from the European Commission. Our NDA submission may receive a refusal to file response from the FDA, and even if filed by the FDA, we may receive a Complete Response Letter rather than approval for commercial marketing. In addition, we may be required by the FDA to conduct additional clinical trials and/or nonclinical studies to support potential approval. 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 cGMPs;
- insufficient data collected from clinical trials of mavorixafor or any other product candidate, 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.

We depend on license agreements with Genzyme, Beth Israel Deaconess Medical Center, Georgetown University and Dana-Farber Cancer Institute 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, Georgetown University and Dana-Farber Cancer Institute 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.

Our license agreement with the Dana-Farber Cancer Institute ("DFCI") imposes upon us various diligence, payment and other obligations, including our obligations to pay DFCI milestone payments in the aggregate amount of up to approximately $32 million, contingent upon our achievement of certain regulatory and sales milestones with respect to licensed products, to deliver reports at regular intervals and to maintain certain minimum levels of insurance. DFCI may terminate the agreement if (i) we cease to carry on our business with respect to the licensed products, (ii) we default on diligence, insurance, payment or any other material obligations, (iii) one of our officers or that of a sublicensee is convicted of a felony relating to the manufacture, use, sale or importation of one or more licensed product, (iv) we become insolvent, (v) we grant a sublicense without notifying DFCI or on terms inconsistent with the terms required of sublicenses under the agreement or (vi) we bring a patent challenge against the licensed products. We have the right to terminate the agreement for any reason upon 90 days advance written notice.

Disputes may arise under any of our license agreements with Genzyme, Beth Israel Deaconess Medical Center, Georgetown University and/or Dana-Farber Cancer Institute 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 safety and/or efficacy of our 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.

Product development involves a lengthy and expensive process, with uncertain outcomes. Delays in or failure to complete any of 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.
To receive the required approval to commercialize any product candidates, we must demonstrate through extensive clinical trials that our product candidates are safe and effective in humans. Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. We may be unable to establish clinical endpoints that applicable regulatory authorities would consider clinically meaningful, and a clinical trial can fail at any stage of testing. Clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in

clinical trials have nonetheless failed to receive marketing approval of their product candidates.

In addition, we may experience delays in our current or future clinical trials, including our Phase 2 clinical trial of mavorixafor for the treatment of chronic neutropenic disorders. For example, as a result of the COVID-19 pandemic, we experienced delays in clinical trial site activation and slower patient enrollment in our clinical trials of mavorixafor for the treatment of WHIM syndrome, Waldenström's and chronic neutropenia disorders. Clinical trials may be delayed, suspended or terminated for a variety of reasons, including the following:

- 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 ("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 ("IRB") approval to conduct a clinical trial at each site;
- delays resulting from negative or equivocal findings of the Data Safety Monitoring Board ("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 supply of drug product for use in nonclinical studies or clinical trials;
- lack of adequate funding to continue the product development program;
- external business disruptions affecting the initiation, patient enrollment, development and operation of our clinical trials, including a public health emergency and unforeseen events such as the war in Ukraine; or
- changes in governmental regulations or requirements.

Any delays in completing or failures to complete 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.

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.

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, enroll and retain patients in testing our product candidates, and we have made certain assumptions about the rate at which we can enroll patients in our clinical trials. 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. For example, as a result of the COVID-19 pandemic, we previously have experienced a slower enrollment pace in some of our clinical trials.

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, 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 current and future clinical trials in a timely manner. In particular, we are currently evaluating mavorixafor for the treatment of chronic neutropenic disorders, which are 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. 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 for mavorixafor in WHIM syndrome and other chronic neutropenic disorders is smaller than we anticipate, our potential future revenue from mavorixafor for the treatment of any of these 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. Our lead clinical candidate, mavorixafor, is being developed as an oral, once-daily therapy for the potential treatment of WHIM syndrome and other chronic neutropenic disorders. We have completed a pivotal, Phase 3 clinical trial (the "4WHIM trial") in people with WHIM syndrome, and are currently advancing mavorixafor in a Phase 2 clinical trial in people with certain chronic neutropenic disorders. We are currently 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 broad online survey of physicians to validate current prevalence estimates and additional research using artificial intelligence, which interrogated a database of more than 300 million anonymized patient records that spanned 10 years of insurance claims, we estimate there are up to 3,700 diagnosed and undiagnosed WHIM patients in the United States, many of whom were previously undiagnosed. If the commercial opportunity in any of our target indications, including 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 and other chronic neutropenic disorders. Our projections of the number of people who have WHIM syndrome (or its other potential primary immunodeficiencies) and chronic neutropenic disorders 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 if mavorixafor is approved for the treatment of WHIM syndrome. 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.

Interim top-line and preliminary data from our clinical trials as well as results of earlier clinical trials may not be predictive of the results of later-stage clinical trials.

The results of pre-clinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. Interpretation of results from early, usually smaller, trials that suggest positive trends in some subjects, require caution. Results from later-stage clinical trials enrolling more subjects may fail to show the desired safety and efficacy results or otherwise fail to be consistent with the results of earlier trials of the same product candidate. Inconsistencies may occur for a variety of reasons, including differences in trial design, trial endpoints (or lack of trial endpoints in exploratory studies), subject population, number of subjects, subject selection criteria, trial duration, drug dosage and formulation or lack of statistical power in the earlier trials.

Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit, or prevent regulatory approval. In addition, we may experience regulatory delays or rejections as a result of many factors, including due to changes in regulatory policy during the period of our product candidate development. Any such delays could negatively impact our business, financial condition, results of operations and prospects.

Interim top-line and preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publish interim top-line or preliminary data from our clinical trials. Interim data from clinical trials are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Preliminary or top-line data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. Preliminary or top-line data may include, for example, data regarding a small percentage of the patients enrolled in a clinical trial, and such preliminary data should not be viewed as an indication, belief or guarantee that other patients enrolled in such clinical trial will achieve similar results or that the preliminary results from such patients will be maintained. As a result, interim and preliminary data should be viewed with caution until the final data are available. Differences between preliminary or interim data and final data could significantly harm our business prospects and may cause the trading price of our common stock to fluctuate significantly.

Risks Related to the Marketing and Commercialization of Our Product Candidates

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. Additionally, 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.

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 ("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.

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 other conditions of approval, or contain requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the drug. 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.

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, or any other sanction by a regulatory authority or other governmental entity, may inhibit our ability to commercialize our products and generate revenue.

The FDA and other regulatory agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. If we are found or alleged to have improperly promoted off-label uses, we may become subject to significant liability.

The FDA and other regulatory agencies strictly regulate the promotional claims that may be made about drug products. These regulations include standards and restrictions for direct-to-consumer advertising, industry-sponsored scientific and educational activities, promotional activities involving the internet and off-label promotion. Any regulatory approval that the FDA grants is limited to those indications and patient populations for which a drug is deemed to be safe and effective by the FDA.

While physicians in the United States may choose, and are generally permitted, to prescribe products for uses that are not described in the product's labeling and for uses that differ from those tested in clinical trials and approved by the regulatory authorities, our ability to promote any of our products candidates, if approved, will be limited to those indications and populations that are specifically approved by the FDA or such other regulatory agencies, and if we are found to have promoted such off-label uses, we may become subject to significant liability. For example, the federal government has levied large civil and criminal fines against companies for alleged improper promotion and in some instances has also required companies to enter into corporate integrity agreements under which specified promotional conduct is changed or curtailed. If we cannot successfully manage the promotion of our product candidates, if approved, we could become subject to significant liability, which would materially adversely affect our business and financial condition.

A Breakthrough Therapy designation or Fast Track designation by the FDA for our product candidates may not lead to a faster development or regulatory review or approval process, and neither of these designations increases the likelihood that our product candidates will receive marketing approval.

We have obtained both Breakthrough Therapy and Fast Track designations for mavorixafor for the treatment of adult patients with WHIM and we may pursue those designations 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. 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.

If a drug is intended for the treatment of a serious or life-threatening condition or disease and the drug demonstrates the potential to address unmet medical needs for the condition, the sponsor may apply for Fast Track designation.

Breakthrough Therapy and Fast Track designations are within the discretion of the FDA. Accordingly, even if we believe that our product candidates meet the criteria for designation, the FDA may disagree and instead determine not to make such designation. The receipt of Breakthrough Therapy designation or Fast Track 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 for Breakthrough Therapies or Fast Track designation, the FDA may later decide that a product candidate no longer meets the conditions for the designation and rescind the designation.

It is possible that we may not be able to obtain or maintain orphan drug designation or exclusivity for our drug candidates, which could limit the potential profitability of our product candidates.

Regulatory authorities in some jurisdictions, including the United States and European Union, 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, 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. We also received orphan drug designation in the U.S. for mavorixafor for the treatment of Waldenström's macroglobulinemia in June 2022. 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. In the European Union, the applicable period is 10 years, during which no marketing authorization may be granted for a similar medicinal product to the authorized orphan product for the same indication. The exclusivity period in the European Union can be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan designation, including if the drug is sufficiently profitable so that marketing exclusivity is no longer justified. Orphan drug exclusivity may be lost in both the United States and European Union under certain limited 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.

The FDA has granted rare pediatric disease designation for mavorixafor for the treatment of WHIM syndrome, however, there is no guarantee that FDA approval of mavorixafor for WHIM will result in a priority review voucher.
In 2012, Congress authorized the FDA to award priority review vouchers to sponsors of certain rare pediatric disease product applications. This program is designed to encourage development of new drug and biological products for prevention and treatment of certain rare pediatric diseases. Specifically, under this program, a sponsor who receives an approval for a drug or biologic for a "rare pediatric disease" that meets certain criteria may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product. The sponsor of a rare pediatric disease drug product receiving a priority review voucher may transfer (including by sale) the voucher to another sponsor. The voucher may be further transferred any number of times before the voucher is used, as long as the sponsor making the transfer has not yet submitted the application. The FDA may also revoke any priority review voucher if the rare pediatric disease drug for which the voucher was awarded is not marketed in the U.S. within one year following the date of approval.

The FDA has granted rare pediatric disease designation for mavorixafor for the treatment of WHIM syndrome; however, there is no guarantee that we will be able to obtain a priority review voucher, even if mavorixafor is approved by the FDA. Under the current statutory provisions, FDA may not award a rare pediatric disease priority review voucher to sponsors of marketing applications unless the drug has received rare pediatric disease designation as of September 30, 2024 and is approved by the FDA no later than September 30, 2026. Even though we received rare pediatric disease designation by the current statutory deadline of September 30, 2024 we may not receive the voucher if we do not obtain approval by September 2026. It is possible that Congress may extend the date by which a rare pediatric disease-designed drug must obtain approval in order to receive a priority review voucher, but even if such legislation is enacted, we may not obtain approval by that date, and even if we do, we may not obtain a priority review voucher.

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 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 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.

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.

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.

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 that 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. Some of these competitive products and therapies are based on scientific approaches that are the same as or similar to our approach and others are based on entirely different approaches. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

We are developing our lead product candidate, mavorixafor, for the treatment of WHIM syndrome and other chronic neutropenic disorders. We are aware of other companies that are developing CXCR4 inhibitors that are in a similar stage of development as mavorixafor, including 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 chronic neutropenia disorders. With respect to chronic neutropenia, filgrastim injections (human granulocyte colony-stimulating factor (G-CSF)) and two biosimilars (Zarxio

and Nivestym) are FDA-approved to reduce the incidence and duration of after affects of severe neutropenia (e.g. , fever, infections, oropharyngeal ulcers) in symptomatic patients with congenital neutropenia, cyclic neutropenia or idiopathic neutropenia.

In many diseases, 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 any of 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.

Even if we obtain and maintain approval for our product candidates from the FDA, we may never obtain approval for our product candidates outside of the United States, which would limit our market opportunities and could harm our business.
Approval of a product candidate in the United States by the FDA does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions, and approval by a foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Even if the FDA grants marketing approval for a product candidate, comparable foreign regulatory authorities also must approve the manufacturing and marketing of the product candidate in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those in the United States, including additional preclinical studies or clinical trials. In many countries outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for any product candidates, if approved, is also subject to approval. Obtaining approval for any future product candidates in the European Union from the European Commission following the opinion of the EMA would be a lengthy and expensive process. Even if a product candidate is approved, the FDA or the European Commission, as the case may be, may limit the indications for which the drug may be marketed, require extensive warnings on the drug labeling or require expensive and time-consuming additional clinical trials or reporting as conditions of approval. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of any future product candidates in certain countries.

If we seek approval to commercialize our product candidates outside of the United States, a variety of risks associated with international operations could harm our business.
If we seek approval of our product candidates outside of the United States, we expect that we will be subject to additional risks in commercialization including:

- different regulatory requirements for approval of therapies in foreign countries;
- reduced protection for intellectual property rights;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;

- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- foreign reimbursement, pricing and insurance regimes;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical actions, including war and terrorism, or natural disasters and public health epidemics.

We have no prior experience in these areas. In addition, there are complex regulatory, tax, labor and other legal requirements imposed by many of the individual countries in and outside of Europe with which we will need to comply. Many biopharmaceutical companies have found the process of marketing their own products in foreign countries to be very challenging.

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. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

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 Government Regulation

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 significant penalties, including administrative, civil and criminal 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 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 research, as well as market, sell and distribute any products for which we obtain marketing approval. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, structuring and commission(s), certain customer incentive programs and other business arrangements generally. Activities subject to these laws also involve the improper use of information received in the course of patient recruitment for clinical trials. See the section in this Annual Report on Form 10-K for the fiscal year ended December 31, 2023 entitled "Business – Government Regulation – Other Healthcare Laws and Compliance Requirements."

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices 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 from government funded healthcare programs, such as Medicare and Medicaid, additional reporting requirements and/or oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws and the curtailment or restructuring of our operations. If any of the physicians or other 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.

We cannot predict what healthcare reform initiatives may be adopted in the future. However, 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. These new laws may result in additional reductions in Medicare and other healthcare funding. 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.

See the sections of this Annual Report on Form 10-K for the fiscal year ended December 31, 2023 entitled, "Business – Government Regulation – Pharmaceutical Coverage, Pricing and Reimbursement" and "Business – Government Regulation – Healthcare Reform."

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 ("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.

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 Dependence on Third Parties

We have no 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 the active pharmaceutical ingredient ("API") for mavorixafor, 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.

To meet our projected needs for clinical supplies to support our development activities through regulatory approval and commercial manufacturing, the manufacturers 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 manufacturers 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, where applicable. 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 of 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, CMOs 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, CMOs 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.

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.

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 changes over the past ten years to the patent laws and to the rules of the United States Patent and Trademark Office ("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 ("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. 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.

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.

As another example, the complexity and uncertainty of European patent laws have increased in recent years. In Europe, a new unitary patent system will likely be introduced by the end of 2023, which would significantly impact European patents, including those granted before the introduction of such a system. Under the unitary patent system, European applications will soon have the option, upon grant of a patent, of becoming a Unitary Patent which will be subject to the jurisdiction of the Unitary Patent Court ("UPC"). As the UPC is a new court system, there is no precedent for the court, increasing the uncertainty of any litigation. Patents granted before the implementation of the UPC will have the option of opting out of the jurisdiction of the UPC and remaining as national patents in the UPC countries. Patents that remain under the jurisdiction of the UPC will be potentially vulnerable to a single UPC-based revocation challenge that, if successful, could invalidate the patent in all countries who are signatories to the UPC. We cannot predict with certainty the long-term effects of any potential changes.

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

Our future success depends on our ability to retain executives and to attract, retain and motivate key personnel in a competitive environment for skilled biotechnology 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 also highly dependent upon members of our current management team, including Paula Ragan, Ph.D., our Chief Executive Officer. The loss of the services provided by these individuals will adversely impact the achievement of our objectives. These individuals could leave our employment at any time, as they are "at will" employees. Effective succession planning is also important to our long-term success. Failure to ensure effective transfer of knowledge and smooth transitions involving key employees could hinder our strategic planning and execution. While we expect to engage in an orderly transition process if and when we integrate newly appointed officers and managers, we face a variety of risks and uncertainties relating to management transition, including diversion of management attention from business concerns, failure to retain other key personnel, or loss of institutional knowledge. In addition, the loss of the services of any of our executive officers, other key employees, and other scientific and medical advisors, and an inability to find suitable replacements could result in delays in product development, and harm our business.

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, 2023, we had 93 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. Additionally, 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.

In addition, we have implemented a work model that has enabled substantially all of our employees to periodically work remotely, which may make us more vulnerable to cyberattacks. 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 selling, 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.

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

Our net operating loss ("NOL") carryforwards could expire unused and be unavailable to offset future tax liabilities because of their limited duration or because of restrictions under U.S. tax law. As of December 31, 2023, we had U.S. federal and state NOLs of $400.0 million and $389.0 million, respectively. Our NOLs generated in tax years ending on or prior to December 31, 2017 are only permitted to be carried forward for 20 years under applicable U.S. tax law. Under the Tax Act, as modified by the CARES

Act, our federal NOLs generated in tax years ending after December 31, 2017 may be carried forward indefinitely, but the deductibility of federal NOLs, particularly for tax years beginning after December 31, 2020, may be limited. It is uncertain if and to what extent various states will conform to the Tax Act and the CARES Act.

Section 382 of the Internal Revenue Code of 1986, as amended, or Section 382, contains rules that limit the ability of a company that undergoes an ownership change to utilize its net operating losses, or NOLs, and tax credits existing as of the date of such ownership change. Under the rules, such an ownership change is generally any change in ownership of more than 50% of a company's stock within a rolling three-year period. The rules generally operate by focusing on changes in ownership among stockholders considered by the rules as owning, directly or indirectly, 5% or more of the stock of a company and any change in ownership arising from new issuances of stock by the company. We have experienced multiple ownership changes since our inception and are conducting a study to assess whether an ownership change has occurred and whether these ownership changes will limit the future use of our NOL carryforwards. Future ownership changes as defined by Section 382 may further limit the amount of NOL carryforwards that could be utilized annually to offset future taxable income.

Our term loan contains restrictions that limit our flexibility in operating our business.
In October 2018, we entered into a loan and security agreement, as most recently amended in August 2023, with Hercules, secured by a lien on substantially all of our assets, excluding 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 a requirement that we maintain cash in an aggregate amount greater than or equal to $20 million; provided through January 31, 2025; provided however that on or after January 31, 2025, such amount must equal 20% of the aggregate principal amount of loans outstanding under the loan and security agreement. Based on our current cash, cash equivalents and marketable securities and our cash flow projections, excluding any gross profit related to the potential sale of our drug, should the FDA approve our current NDA, and excluding the potential sale of any priority review voucher that might be received upon approval of the Company's NDA noted above, and with no additional borrowings under our existing debt facility or other sources of external financing, we believe that we would be in violation of the minimum cash described above in the first quarter of 2025. 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.

Our business could be adversely affected by economic downturns, inflation, increases in interest rates, natural disasters, public health crises, political crises, geopolitical events, such as the war in Ukraine and in Gaza, or other macroeconomic conditions, which have in the past and may in the future negatively impact our business and financial performance.
The global economy, including credit and financial markets, has experienced extreme volatility and disruptions, including, among other things, severely diminished liquidity and credit availability, declines in consumer confidence, declines in economic growth, supply chain shortages, increases in inflation rates, higher interest rates and uncertainty about economic stability. For example, the COVID-19 pandemic resulted in widespread unemployment, economic slowdown and extreme volatility in the capital markets. The U.S. Federal Reserve recently raised interest rates multiple times in response to concerns about inflation and it may raise them again. Higher interest rates, coupled with reduced government spending and volatility in financial markets may increase economic uncertainty. If the equity and credit markets deteriorate, including as a result of political unrest or war, such as the war in Ukraine or in Gaza, it may make any necessary debt or equity financing more difficult to obtain in a timely manner or on favorable terms, more costly or more dilutive. Increased inflation rates can adversely affect us by increasing our costs, including labor and employee benefit costs.

Risks Related to Ownership of Our Common Stock

We are currently not in compliance with the Nasdaq continued listing requirements. If we are unable to regain compliance with Nasdaq's listing requirements, our securities could be delisted, which could affect our common stock's market price and liquidity and reduce our ability to raise capital.

On November 13, 2023, we received a letter from the Listing Qualifications Staff of The Nasdaq Stock Market LLC ("Nasdaq") indicating that, based upon the closing bid price of our common stock for the last 30 consecutive business days, we no longer meet Nasdaq Listing Rule 5550(a)(2), which requires listed companies to maintain a minimum bid price of at least $1.00 per share.

In accordance with the listing rules of Nasdaq, the Company has been given 180 calendar days, or until May 13, 2024 (the "Compliance Date"), to regain compliance with the minimum bid price requirement. If at any time before the Compliance Date, the closing bid price of the Company's Common Stock is at least $1.00 per share for a minimum of ten consecutive business days, Nasdaq will provide written notification to the Company that it complies with the minimum bid price requirement. If the Company is unable to regain compliance before the Compliance Date, the Company may be eligible for an additional 180 calendar days to satisfy the Bid Price Rule. To qualify, the Company will be required to meet the continued listing requirement for market value of publicly held shares and all other initial listing standards for the Nasdaq Capital Market with the exception of the Bid Price Rule, and will need to provide written notice of its intention to cure the deficiency during such additional compliance period, by effecting a reverse stock split, if necessary. If it appears to Nasdaq staff that the Company will not be able to cure the deficiency, or if the Company is otherwise not eligible for the additional compliance period, and the Company does not regain compliance by the Compliance Date, Nasdaq will provide written notification to the Company that its Common Stock is subject to delisting. At that time, the Company may appeal the delisting determination to a hearings panel pursuant to the procedures set forth in the applicable Nasdaq Listing Rules. However, there can be no assurance that, if the Company does appeal the delisting determination by Nasdaq to the panel, such appeal would be successful.

If we fail to regain compliance with the Nasdaq continued listing standards, Nasdaq will provide notice that our common stock will be subject to delisting. We would then be entitled to appeal that determination to a Nasdaq hearings panel.

The notification has no immediate effect on the listing of our common stock on Nasdaq. We intend to monitor the closing bid price of our common stock and consider our available options in the event the closing bid price of our common stock remains below $1.00 per share.

We cannot assure you that we will be able to regain compliance with Nasdaq listing standards. Our failure to continue to meet the minimum bid requirement would result in our common stock being delisted from Nasdaq. We and holders of our securities could be materially adversely impacted if our securities are delisted from Nasdaq. In particular:

- we may be unable to raise equity capital on acceptable terms or at all;
- we may lose the confidence of our customers, which would jeopardize our ability to continue our business as currently conducted;
- the price of our common stock will likely decrease as a result of the loss of market efficiencies associated with Nasdaq and the loss of federal preemption of state securities laws;
- holders may be unable to sell or purchase our securities when they wish to do so;
- we may become subject to stockholder litigation;
- we may lose the interest of institutional investors in our common stock;
- we may lose media and analyst coverage;
- our common stock could be considered a "penny stock," which would likely limit the level of trading activity in the secondary market for our common stock; and
- we would likely lose any active trading market for our common stock, as it may only be traded on one of the over-the-counter markets, if at all.

Our stock price has been and is likely to continue to be volatile and fluctuate substantially.

The market price of our common stock has been and could continue to be subject to significant fluctuations. Market prices for securities of 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 macroeconomic, political and market conditions and overall fluctuations in the financial markets in the United States and abroad;
- 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;
- period-to-period fluctuations in our financial results; and
- the other factors described in this “Risk Factors” section and elsewhere in this Annual Report

In addition, companies trading in the stock market in general have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies, which has resulted in decreased stock prices for many companies notwithstanding the lack of a fundamental change in their underlying business models or prospects. Broad market and industry factors, including potentially worsening economic conditions and other adverse effects, may negatively affect the market price of our common stock, regardless of our actual operating performance. In the past, following periods of volatility in the market 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.

"Penny stock" rules may make buying or selling our securities difficult which may make our stock less liquid and make it harder for investors to buy and sell our securities.

Trading in our securities is subject to the SEC's "penny stock" rules and it is anticipated that trading in our securities will continue to be subject to the penny stock rules for the foreseeable future. The SEC has adopted regulations that generally define a penny stock to be any equity security that has a market price of less than $5.00 per share, subject to certain exceptions. These rules require that any broker-dealer who recommends our securities to persons other than prior customers and accredited investors must, prior to the sale, make a special written suitability determination for the purchaser and receive the purchaser's written agreement to execute the transaction. Unless an exception is available, the regulations require the delivery, prior to any transaction involving a penny stock, of a disclosure schedule explaining the penny stock market and the risks associated with trading in the penny stock market. In addition, broker-dealers must disclose commissions payable to both the broker-dealer and the registered representative and current quotations for the securities they offer. The additional burdens imposed upon broker-dealers by these requirements may discourage broker-dealers from recommending transactions in our securities, which could severely limit the liquidity of our securities and consequently adversely affect the market price for our securities.

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 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.

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.

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 Securities Exchange Act of 1934, as amended (the "Exchange Act"), the Sarbanes-Oxley Act of 2002 and the rules and regulations of The Nasdaq Stock Market ("Nasdaq"). Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002 ("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 Annual Report.

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, 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

consolidated 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 a non-accelerated filer, 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 a smaller reporting company and no longer qualify as a non-accelerated filer, 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 consolidated 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 consolidated financial statements, the market price of our stock could decline and we could be subject to sanctions or investigations by Nasdaq, the SEC or other regulatory authorities.

We are a "smaller reporting company" and cannot predict if the reduced reporting requirements applicable to smaller reporting companies will make our securities less attractive to investors.
We are a "smaller reporting company" under the Exchange Act as of June 30, 2023. We may continue to be a smaller reporting company if either (i) the market value of our common stock held by non-affiliates is less than $250 million or (ii) our annual revenue was less than $100.0 million during the most recently completed fiscal year and the market value of our common stock held by non-affiliates is less than $700.0 million. As a smaller reporting company, we may rely on exemptions from certain disclosure requirements that are available to smaller reporting companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. For so long as we remain a smaller reporting company, we are permitted and intend to rely on such exemptions from certain disclosure and other requirements that are applicable to other public companies that are not smaller reporting companies.

We cannot predict if investors will find our securities less attractive because we may rely on the exemptions and reduced disclosure obligations applicable to smaller reporting companies. If some investors find our securities 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.

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. The outcome of litigation is necessarily uncertain, and we could be forced to expend significant resources in the defense of such suits, and we may not prevail. Monitoring and defending against legal actions is time-consuming for our management and detracts from management's ability to fully focus our internal resources on our business activities. In addition, we may incur substantial legal fees and costs in connection with any such litigation. We have not established any reserves for any potential liability relating to any such potential lawsuits. It is possible that we could, in the future, incur judgments or enter into settlements of claims for monetary damages. We currently maintain insurance coverage for some of these potential liabilities. Other potential liabilities may not be covered by insurance, insurers may dispute coverage or the amount of insurance may not be enough to cover damages awarded. In addition, certain types of damages may not be covered by insurance, and insurance coverage for all or certain forms of liability may become unavailable or prohibitively expensive in the future. A decision adverse to our interests on one or more legal matters or litigation could result in the payment of substantial damages, or possibly fines, and could have a material adverse effect on our reputation, financial condition and results of operations.

Provisions in our corporate charter documents and under Delaware law could make an acquisition of our 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 our 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 1C. CYBERSECURITY

Cybersecurity Risk Management and Strategy

Our management recognizes the impact that cybersecurity threats could have on our business operations, our compliance with regulations, and our reputation. We have identified cybersecurity as a critical business risk as part of our overall risk management strategy, which our board of directors oversees.

We have implemented a cybersecurity program in accordance with our risk profile and business that includes, among other things, written policies, monitoring and filtering procedures, and employee training. We have also developed an incident response policy and procedure designed to facilitate the timely reporting and assessment of cybersecurity incidents.

Our cybersecurity risk management program, which is part of our enterprise risk management program, aims to identify risks related to the Company, including risks from cybersecurity threats. Our cybersecurity risk management program includes a number of components, including informal self-assessments and audits, penetration testing, and vulnerability assessments, that are conducted periodically by both internal and external resources. The Company also analyzes current and emerging cyber threats that pose a risk to the organization using various threat intelligence tools and services.

As part of our cybersecurity risk management program, we take a risk-based approach to the evaluation of third-party vendors, and apply mitigations and processes based on our evaluation of the sensitivity of the data accessed by the vendor and the maturity of the vendor's programs. Our vendor evaluation procedures include, as appropriate, the review of vendors' SOC 2 Type 2 reports if available, and a vendor security questionnaire. We are in the process of expanding the use of the security questionnaire to additional vendors.

Governance Related to Cybersecurity Risks

Our director of information technology ("Director of IT") is responsible for the strategic leadership and direction of the Company's information technology organization. The Director of IT has helped organizations define and implement information technology strategies for over twenty years. Prior to joining the Company, he served in senior information technology roles for several biotechnology companies. Along with members of our finance, legal, and operations teams, the Director of IT sits on a newly-formed cyber subcommittee ("subcommittee"). The subcommittee reports to the Chief Operating Officer ("COO") and Chief Financial Officer ("CFO"), and outputs from the Committee are provided by the COO and CFO to the Company's executive team and the board.

The board is responsible for informed oversight of our risk management process. The board administers this oversight function through various board standing committees that address risks inherent in their respective areas of oversight. The board has delegated oversight for cybersecurity risk management to the Audit Committee. The Audit Committee reviews the Company's policies and procedures with respect to cybersecurity risk management.

Although risks from cybersecurity threats have to date not materially affected us, our business strategy, results of operations or financial condition, we have, from time to time, experienced threats to and breaches of our and our third-party vendors' data and systems. For more information, see Item 1A. Risk Factors. ***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.***

ITEM 2. PROPERTIES

We lease approximately 28,000 square feet of office space at 61 North Beacon Street, 4th Floor, Boston, Massachusetts, which serves as our corporate headquarters. The lease expires on November 30, 2026. The base monthly payment on the lease is approximately $91 thousand as of December 31, 2023, subject to specified annual increases of approximately 3% during the term of the lease and not including operating expenses, certain utilities, taxes and insurance for which we are responsible. 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.

We lease approximately 1,200 square meters of laboratory and office space in Vienna, Austria under a lease that will expire in March 2028, with a monthly payment of approximately $24 thousand.

ITEM 3. LEGAL PROCEEDINGS

From time to time, we may be involved in lawsuits, claims, investigations and proceedings, consisting of intellectual property, commercial, employment and other matters which arise in the ordinary course of business. While the outcome of any such proceedings cannot be predicted with certainty, as of December 31, 2023, we were not party to any 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 14, 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 Capital Market under the symbol "XFOR".

Holders of Our Common Stock

As of March 1, 2024, there were 60 holders of record of our common stock, one of which is Cede & Co., a nominee for Depository Trust Company ("DTC"). All of the shares of common stock held by brokerage firms, banks and other financial institutions as nominees for beneficial owners are deposited into participant accounts at DTC, and are considered to be held of record by Cede & Co. as one stockholder.

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.

Equity Compensation Plan

The information required by Item 5 of Form 10-K regarding equity compensation plans is incorporated herein by reference to Item 12 of Part III of this Annual Report on Form 10-K.

Recent Sales of Unregistered Securities

None.

Purchase of Equity Securities by the Issuer and Affiliated Purchasers

None.

ITEM 6. [RESERVED]

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, 2022 compared to the year ended December 31, 2021, refer to "Management's Discussion and Analysis of Financial Condition and Results of Operations" to our Annual Report on Form 10-K filed with the SEC on March 21, 2023.

Overview

We are a late clinical-stage biopharmaceutical company discovering and developing novel therapeutics for the treatment of rare diseases and those with limited treatment options, with a focus on conditions resulting from dysfunction of the immune system.

Our lead clinical candidate is mavorixafor, a small-molecule selective antagonist of chemokine receptor CXCR4 that is being developed as an oral, once-daily therapy. Due to its ability to increase the mobilization of mature, functional white blood cells into the bloodstream, we believe that mavorixafor has the potential to provide therapeutic benefit across a variety of chronic neutropenic disorders, including WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) syndrome, a rare, primary immunodeficiency for which there are no approved therapies.

We are currently seeking approval from the U.S. Food and Drug Administration ("FDA") for the use of oral, once-daily mavorixafor in the treatment of people aged 12 years and older with WHIM syndrome following the October 2023 acceptance of our New Drug Application ("NDA") by the FDA. The FDA has granted the NDA Priority Review, establishing a goal of six months review from the date of acceptance and assigning a Prescription Drug User Fee Act ("PDUFA") target action date of April 30, 2024. At this time, the FDA has notified us that they are not currently planning to hold an advisory committee meeting to review the filing. Due to mavorixafor's Rare Pediatric Disease designation in the U.S. for WHIM syndrome, we are eligible to receive a Priority Review Voucher ("PRV") that can be used to obtain Priority Review for a subsequent application or sold to another drug sponsor should mavorixafor be approved.

The NDA is supported by our successfully completed global, pivotal, Phase 3 clinical trial (4WHIM) that evaluated the safety and efficacy of mavorixafor in people with WHIM syndrome. The 4WHIM trial met its primary endpoint and a key secondary endpoint, demonstrating statistically significant increases in time above threshold for absolute neutrophil counts ("TAT-ANC") and time above threshold for absolute lymphocyte counts ("TAT-ALC") in participants treated with mavorixafor versus placebo. Additional data showed that mavorixafor treatment resulted in statistically significant reductions in annualized infection rates versus placebo and clinically meaningful reductions in the both the severity and duration of infections versus placebo. Mavorixafor was generally well tolerated throughout the 52-week trial.

In anticipation of a potential second quarter of 2024 U.S. launch of mavorixafor in WHIM syndrome, we have continued to build our go-to-market organization, with key hires across commercial and medical functions, increased interactions with key stakeholders and rare disease patient advocacy organizations, and launched a disease-awareness campaign aiming to further the understanding of WHIM syndrome and educate patients and physicians on the importance and benefits of early diagnosis.

We are also currently advancing mavorixafor for the treatment of people with certain chronic neutropenic disorders following positive results from a Phase 1b clinical trial of a single dose of mavorixafor in people with idiopathic, cyclic, and congenital chronic neutropenia. We are now conducting a Phase 2 clinical trial, evaluating the durability, safety, and tolerability of chronic dosing of once-daily oral mavorixafor with or without concurrent treatment with injectable granulocyte colony-stimulating factor ("G-CSF") in the same patient population. Preliminary results from the trial showed that the first three participants experienced clinically meaningful increases in ANC. We expect to share further data from the Phase 2 trial in the second quarter of 2024. Concurrent with conducting this Phase 2 trial, we are advancing our plans for a Phase 3 trial in the first half of 2024 of mavorixafor in people with certain chronic neutropenic disorders. The planned 52-week, global Phase 3 trial is expected to be a

randomized, double-blinded, placebo-controlled trial assessing the safety and efficacy of mavorixafor, with or without concomitant G-CSF, in people with idiopathic or congenital neutropenia.

We believe that successfully developing mavorixafor and providing a new therapeutic option to individuals diagnosed with certain chronic neutropenic disorders has the potential to revolutionize the treatment landscape, which is principally served by injectable therapies (including G-CSF) that are frequently associated with treatment-limiting adverse events.

To date, we have not generated revenue from product sales. 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.

Macroeconomic Considerations

Unfavorable conditions in the economy in the United States and abroad may negatively affect the growth of our business and our results of operations. The effect of macroeconomic conditions may not be fully reflected in our results of operations until future periods. If, however, economic uncertainty increases or the global economy worsens, our business, financial condition and results of operations may be harmed. For further discussion of the potential impacts of macroeconomic events on our business, financial condition, and operating results, see the section titled "Risk Factors."

Results of Operations

Comparison of the Years Ended December 31, 2023 and 2022

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

	Year Ended December 31,		
	2023	2022	Change
	(in thousands)		
Operating expenses:			
Research and development	$ 72,017	$ 61,058	$ 10,959
Selling, general and administrative	35,505	27,020	8,485
Gain on sale of non-financial asset	—	(509)	509
Total operating expenses	107,522	87,569	19,953
Loss from operations	(107,522)	(87,569)	(19,953)
Total other income (expense), net	6,433	(6,270)	12,703
Loss before provision for income taxes	(101,089)	(93,839)	(7,250)
Provision for income taxes	78	28	50
Net loss	$ (101,167)	$ (93,867)	$ (7,300)

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 ("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 ("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,		
	2023	2022	Change
	(in thousands)		
Direct research and development expenses by product candidate:			
Mavorixafor (X4P-001)	$ 41,163	$ 30,041	$ 11,122
X4P-002	(34)	2,338	(2,372)
X4P-003	75	206	(131)
Unallocated expense	30,813	28,473	2,340
Total research and development expenses	$ 72,017	$ 61,058	$ 10,959

Research and development expenses were $72.0 million for the year ended December 31, 2023, as compared to $61.1 million for the year ended December 31, 2022, reflecting an increase of $11.0 million. The increase in research and development expenses in 2023 as compared to 2022 was primarily due to a $5.0 million development milestone payment under our Genzyme agreement, approximately $2 million of higher regulatory costs associated with the preparation and submission of our NDA, and additional consulting and temporary help across our research and development organization to support our NDA submission and our ongoing clinical trials. Research and development expenses also increased in 2023 due to an increase in unallocated expenses, primarily due to an increase in head count within our manufacturing, regulatory and clinical operations functions, resulting in higher compensation expenses, including stock-based compensation.

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 clinical trials of mavorixafor in chronic neutropenic disorders. Research and development expenses related to our X4P-002 and X4P-003 programs was not significant in 2023 or 2022 relative to our overall research and development expenses.

Selling, General and Administrative Expenses

Selling, general and administrative expenses consist primarily of salaries and related costs, including stock-based compensation for personnel in sales and marketing, executive, finance and administrative functions. Selling, 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.

Selling, general and administrative expenses were $35.5 million in 2023, as compared to $27.0 million in 2022, reflecting an increase of $8.5 million. The increase in selling, general and administrative expenses in 2023 as compared to 2022 was primarily due to an increase in compensation expense due to in increase in head count, higher stock-based compensation costs and higher stock appreciation rights expense. We have added personnel to our marketing and sale operations organizations in anticipation of a potential commercial launch of our drug candidate in 2024. We have also incurred third party consulting costs associated with enabling our market access infrastructure. We expect selling, general and administrative expenses will grow in the future as we continue to build out our selling, general and administrative functions.

Goodwill Impairment

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 or when our market value, measured as the price of our common stock multiplied by shares outstanding drops below the value of our net assets.

We have determined that our company operates in a single operating segment and has a single reporting unit. To perform the quantitative test, we compare the fair value of the reporting unit to its carrying value. As we have one reporting unit, we determine its fair value based on the market approach taking into consideration the market value of the company as a whole and any control premium that might be realized upon the sale of the reporting unit. 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, we measure the amount of impairment loss, if any, as the excess of the carrying value over the fair value of the reporting unit. As of December 31, 2023 we determined that goodwill was not impaired based on its quantitative test.

Gain on Sale of Non-Financial Asset

During the year ended December 31, 2022, a third party, who had previously acquired rights to certain intellectual property from us, terminated the arrangement and transferred these rights back us and we transferred these rights to another third party in return for $0.5 million. We have no continuing involvement in any ongoing research and development activities associated with the intellectual property. We concluded that these third parties are "non-customers" as the underlying intellectual property transferred to and from these third parties supports potential drug candidates that are not aligned with our strategic focus and, therefore, are not an output of our ordinary activities. Accordingly, we classified this transaction as a "gain on sale of non-financial asset" for the year ended December 31, 2022. There was no such transaction in year ended December 31, 2023.

Other Income (Expense), Net

	Year Ended December 31,		
	2023	2022	Change
	(in thousands)		
Interest income	$ 4,582	$ 219	$ 4,363
Interest expense	(5,777)	(3,993)	(1,784)
Change in fair value of warrant and derivative liabilities	7,074	1,701	5,373
Research and development incentive program	553	534	19
Foreign currency losses and issuance costs related to warrants	1	(4,731)	4,732
Total other income (expense), net	$ 6,433	$ (6,270)	$ 12,703

The increase in other income (expense), net, of $12.7 million for the year ended December 31, 2023 as compared to 2022 was primarily due (i) an increase in interest income earned on our marketable securities and cash equivalents due to higher invested balances and higher interest rates (ii) income resulting from the decrease in the fair value of outstanding Class C warrants, which are accounted for as a liability, due primarily to a decrease in the value of our common stock and, (iii) transaction fees incurred in 2022 that did not recur in 2023 associated with the issuance of warrants that are accounted for as liabilities, partially offset by an increase in interest expense associated with the Hercules Loan agreement due to higher borrowings and a higher effective interest rate.

Income Taxes

For the years ended December 31, 2023 and 2022, we recorded income tax provisions related to our Austrian subsidiary and Securities Corp. We do not expect to record a significant income tax benefit or expense for several years as we have significant net operating loss carryforwards that are fully reserved until such time as we begin to generate meaningful taxable income in our U.S. jurisdiction.

Liquidity and Capital Resources

Sources of Liquidity

To date, we have funded our operations 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.

ATM Sales Agreement — We have entered into a Controlled Equity Offering℠ Sales Agreement ("ATM Sales Agreement"), with B. Riley Securities, Inc., Cantor Fitzgerald & Co., and Stifel, Nicolaus & Company, Incorporated (collectively the "Sales Agents"), pursuant to which we may offer and sell, at our sole discretion through one or more of the Sales Agents, shares of our common stock having an aggregate offering price of up to $75 million. To date, we have sold approximately $14.3 million of our common stock, net of offering costs, under the ATM Sales Agreement.

LPC Agreement — In January 2022, we entered into an agreement, (the "LPC Agreement") with Lincoln Park Capital Fund LLC ("Lincoln Park"), pursuant to which we have the right to sell to Lincoln Park shares of our common stock, having an aggregate value of up to $50.0 million, subject to certain limitations and conditions, at our request during a 36-month period. The shares of

common stock that we may sell under the LPC Agreement are capped at an aggregate of 5.6 million shares, which amount may be adjusted under certain conditions as defined in the LPC Agreement. In January 2022, we raised $3.0 million from the sale of shares of our common stock through the LPC Agreement.

Public and Private Equity Offerings — Over the past several years we have funded our operations primarily from sales of common stock, warrants and prefunded warrants through both public offerings and private placements. In March 2022, we sold shares of common stock and, in lieu of common stock, pre-funded warrants to purchase shares of common stock in a private placement for gross proceeds of $3.0 million, before offering expenses. In June 2022, we sold shares of common stock and, in lieu of common stock, pre-funded warrants to purchase shares of common stock in a private placement for gross proceeds of $55.7 million, before offering expenses. In December 2022, we sold shares of common stock and, in lieu of common stock, pre-funded warrants to purchase shares of common stock in a public offering for gross proceeds of $65.1 million, before offering expenses. In May 2023, we sold shares of common stock and, in lieu of common stock, pre-funded warrants to purchase shares of common stock in a private placement for gross proceeds of $65.0 million, before offering expenses.

Hercules Loan Agreement — In January 2023, we entered into a Second Amended and Restated Loan and Security Agreement (the "Second A&R Hercules Loan Agreement") with Hercules Capital, Inc., as agent and lender, and Hercules Capital Funding IV LLC and Hercules Capital Funding Trust 2022-1, as lenders (collectively, "Hercules"). On August 2, 2023, we and Hercules entered into an amendment (the "Amendment") to the Second A&R Hercules Loan Agreement, (as amended by the Amendment, the "Hercules Loan Agreement"), which provides for aggregate maximum borrowings of up to $115.0 million, including the $32.5 million outstanding prior to the Amendment, a $22.5 million term loan tranche drawn on the closing of the Amendment, and $60.0 million in potential additional borrowings. These additional borrowings are available upon the achievement of operational milestones, including $20 million of additional borrowings upon the approval ("Approval") by the FDA of our NDA for mavorixafor for the treatment of WHIM syndrome. Borrowings under the Hercules Loan Agreement accrues interest at a variable rate equal to the greater of (i) 10.15% or (ii) 3.15% plus the Wall Street Journal prime rate and are repayable in monthly interest-only payments through March 1, 2025, and in equal monthly payments of principal and accrued interest from April 1, 2025 until the maturity date of the loans, which is currently October 1, 2026, subject to extension upon our achievement of certain operational milestones. The Hercules Loan Agreement requires that we maintain a minimum level of cash of $20.0 million through January 31, 2025, which amount is subsequently adjusted subject to our achievement of operational milestones.

Going Concern— Since our inception, we have incurred significant operating losses and negative cash flows from our operations. We have not yet commercialized any products. As of December 31, 2023, our cash and cash equivalents were $99.2 million, our short-term marketable securities were $15.0 million and our restricted cash balance was $1.0 million. As noted above, we have a covenant under our Hercules Loan Agreement that currently requires that we maintain a minimum level of cash of $20.0 million through January 31, 2025, subject to subsequent reductions thereafter. Based on our current cash flow projections, which exclude any benefit from the potential approval and sale of our drug candidate, and with no additional borrowings that may become available on Hercules Loan Agreement and with no additional external funding, we believe that we will not be able to maintain the minimum cash required to satisfy this covenant beginning in the first quarter of 2025. In such event, the lenders could require the repayment of all outstanding debt.

Management has concluded that substantial doubt exists about our ability to continue as a going concern for the one-year period following the issuance of our consolidated financial statements for the year ended December 31, 2023. To finance our operations, we will need to raise additional capital, which cannot be assured. Unless and until we reach profitability in the future, we will require additional capital to fund our operations, which could be raised through a combination of equity offerings, debt financings, other third-party funding, marketing and distribution arrangements and other collaborations and strategic alliances. If we are unable to obtain funding, we could be forced to delay, reduce or eliminate some or all of our research and development programs, product portfolio expansion or commercialization efforts, which would adversely affect our business prospects, or we may be unable to continue operations.

Cash Flows

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

	Year Ended December 31,	
	2023	2022
	(in thousands)	
Net loss	$ (101,167)	$ (93,867)
Adjustments to reconcile net loss to net cash used in operating activities	4,311	11,029
Changes in operating assets and liabilities	344	5,736
Net cash used in operating activities	(96,512)	(77,102)
Net cash used in investing activities	(14,883)	(103)
Net cash provided by financing activities	88,516	117,230
Impact of foreign exchange on cash and restricted cash	99	(105)
Net increase in cash, cash equivalents and restricted cash	(22,780)	39,920
Cash, cash equivalents and restricted cash, beginning of period	123,028	83,108
Cash, cash equivalents and restricted cash, end of period	$ 100,248	$ 123,028

Operating Activities: During the year ended December 31, 2023, net cash used in operating activities was $96.5 million, primarily resulting from our net losses of $101.2 million, adjusted for noncash expenses of $4.3 million and changes in our operating assets and liabilities of $0.3 million. Noncash expenses primarily includes stock-based compensation expense of $8.7 million and non-cash lease expense of $1.6 million, partially offset by $7.1 million of non-cash gains on the change in fair value of our Class C Warrant liability. The change in operating assets and liabilities was primarily due to an increase in accounts payable and accrued expenses due to the timing of payments related to our CROs. Cash used in operating activities was higher for the year ended December 31, 2023 as compared to the prior year primarily due to higher net losses in the current year.

Investing Activities: During the year ended December 31, 2023, net cash used in investing activities was $14.9 million, primarily due to our net investment in short-term marketable securities. Cash used in investing activities was not significant for the year ended December 31, 2022.

Financing Activities: During the year ended December 31, 2023, net cash provided by financing activities was $88.5 million, consisting primarily of net proceeds from a private placement of our common stock, warrants, and pre-funded warrants, a $22.5 million term loan tranche drawn on the closing of the Amendment of our Hercules Loan Agreement and the exercise of outstanding warrants during the year. During the year ended December 31, 2022, net cash provided by financing activities was $117.2 million, consisting primarily of proceeds from the sale of our common stock and pre-funded warrants in two a private placements and a public offering.

Material Capital Requirements

Debt Obligations

See Note 7 of the consolidated financial statements and in “Sources of Liquidity” above for a description of our debt obligation.

Lease Obligations

We have long-term lease obligations for office and laboratory space. Non-cancellable lease obligations are $1.4 million in 2024, $1.4 million in 2025, $1.3 million in 2026, and $0.3 million thereafter.

Funding Requirements

We believe that our current cash, cash equivalents and short-term marketable securities will allow us to fund operations into the first quarter of 2025. As noted above, based on our current financial projections and assuming no benefit from the potential FDA approval of our market candidate nor the sale of a PRV, if obtained, or any additional borrowings under our Hercules Loan

Agreement, we believe we would not maintain compliance with the minimum cash covenant of the Hercules Loan Agreement in the first quarter of 2025. In order to fund operations and satisfy the minimum cash covenant in the Hercules Loan Agreement, we will be required to raise additional capital, which may be through a combination of equity offerings, debt financings, other third-party funding, marketing and distribution arrangements, the sale of a PRV that may be granted to us if we receive FDA approval of our NDA, profits generated from the sale of our drug candidate upon the receipt of FDA approval of our NDA and other collaborations and strategic alliances. During 2024 and beyond, assuming no changes to our current operational expectations, we expect our expenses to continue to increase in connection with our ongoing activities, particularly as we advance the current and anticipated clinical trials of our product candidates in development and prepare for the launch and commercialization of any product candidates. 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 funding requirements. Our short term and long term 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 2 clinical trial of mavorixafor for the treatment of patients with chronic neutropenic disorders;
- 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 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;
- market acceptance of our product candidates, to the extent any are approved for commercial sale;
- the effect of competing technological and market developments; and
- the costs to operate as a public company

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. We have effective universal shelf registration statements on Form S-3 registering the sale of our common stock, warrants to purchase our common stock and other securities on terms that we may determine. We have an ATM Sales Agreement with the Sales Agents, pursuant to which we have offered to sell and continue to offer to sell, at our sole discretion through one or more of the Sales Agents, shares of our common stock. We have entered into a common stock purchase agreement with Lincoln Park Capital, pursuant to which Lincoln Park Capital has committed to purchase, at our request from time to time over a 36-month period, shares of our common stock having an aggregate offering price of up to $50.0 million, of which $3.0 million have been sold to date, subject to certain limitations.

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.

Critical Accounting 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. For our significant vendors, we 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. We have also issued stock-based awards with performance-based vesting conditions that vest in part upon our achievement of operational milestones and over time thereafter for the subsequent two years as the employee continues to provide services. We assess the probability of achievement of these operational milestones and recognize stock-based compensation for these awards using the accelerated attribution model based on the fair value of the awards as of the date of grant and our best estimate of the date each operational milestone will be achieved. We update our estimates related to the probability and timing of achievement of the operational milestones each period until the award either vests or is forfeited.

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 an 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.

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.

We have determined that we operate in a single operating segment and have a single reporting unit. To perform its quantitative test, we compare the fair value of our single reporting unit to the carrying value of its net assets, including goodwill. We use our market capitalization (common shares outstanding multiplied by the price per share of our common stock) to measure the fair

value of the reporting unit. 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, we measure the impairment loss as the excess of the carrying value over the fair value of the reporting unit. See Note 4 for more information on our goodwill impairment test as of December 31, 2023.

Smaller Reporting Company Status

We are a smaller reporting company as defined in the Exchange Act. We may take advantage of certain of the scaled disclosures available to smaller reporting companies and will be able to take advantage of these scaled disclosures for so long as (i) our voting and non-voting common stock held by non-affiliates is less than $250.0 million measured on the last business day of our second fiscal quarter or (ii) our annual revenue is less than $100.0 million during the most recently completed fiscal year and our voting and non-voting common stock held by non-affiliates is less than $700.0 million measured on the last business day of our second fiscal quarter.

Recently Issued Accounting Pronouncements

There are no recently issued accounting pronouncements that may potentially cause a material impact our financial position and results of operations.

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 Annual Report.

The financial statements contain a Report of Independent Registered Public Accounting Firm PricewaterhouseCoopers LLP, Boston, Massachusetts, US (Firm ID 238).

An index of those financial statements is found in Item 15 of Part IV of this Annual Report.

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, 2023. 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.

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 Rules 13a-15(f) and 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.

Management conducted an assessment of our internal controls over financial reporting based on the framework established by the Committee of Sponsoring Organizations of the Treadway Commission in *Internal Control-Integrated Framework (2013)*. These criteria are in the areas of control environment, risk assessment, control activities, information and communication, and monitoring. Management's assessment included extensive documentation, evaluating and testing the design and operating effectiveness of its internal controls over financial reporting. Based on the assessment, management concluded that, as of December 31, 2023, our internal control over financial reporting was effective.

Attestation Report of the Registered Public Accounting Firm

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 non-accelerated filers.

Changes in Internal Control over Financial Reporting

There have been no significant changes 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, 2023 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B. OTHER INFORMATION

Rule 10b5-1 Trading Plans

During the three months ended December 31, 2023, none of the Company's directors or officers adopted, materially modified, or terminated any contract, instruction, or written plan for the purchase or sale of Company securities that was intended to satisfy the affirmative defense conditions of Rule 10b5-1(c) or any non-Rule 10b5-1 trading arrangement.

ITEM 9C. DISCLOSURE REGARDING FOREIGN JURISDICTIONS THAT PREVENT INSPECTIONS

Not applicable.

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 Regarding the Board of Directors and Corporate Governance" and "Executive Officers" in our 2024 Proxy Statement.

Information regarding our Code of Business Conduct and Ethics (the "Code of Conduct") required by this item will be contained in our 2024 Proxy Statement under the caption "Information Regarding the Board of Directors and Corporate Governance – Code of Ethics," and is hereby incorporated by reference. If we make any substantive amendments to the Code of Conduct or grant any waiver from a provision of the Code of Conduct to any executive officer or director, we will promptly disclose the nature of the amendment or waiver on our website. The full text of our Code of Conduct is available at the Investor Relations section of our website at investors.x4pharma.com/investor-relations. The reference to our website address does not constitute incorporation by reference of the information contained at or available through our website, and you should not consider it to be a part of this Annual Report on Form 10-K.

We intend to satisfy the disclosure requirement under Item 5.05 of Form 8-K regarding an amendment to, or waiver from, a provision of this Code of Business Conduct and Ethics by posting such information on our website, at the address and location specified above and, to the extent required by the listing standards of The Nasdaq Global Select Market, by filing a Current Report on Form 8-K with the SEC, disclosing such information.

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 2024 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 2024 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 2024 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 2024 Proxy Statement.

PART IV

ITEM 15. EXHIBIT AND FINANCIAL STATEMENT SCHEDULES

(1) Financial Statements

The following documents are included on pages F-1 through F-36 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 consolidated financial statements or the notes thereto.

(3) Exhibits.

Exhibit No.	Exhibit Description	Form	Exhibit	Date	Se File/ Ref No.
3.1	Restated Certificate of Incorporation, as amended, as of September 1, 2022	8-K	3.1	9/1/2022	001-38295
3.2	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	Securities Purchase Agreement, dated March 18, 2021, by and among X4 Pharmaceuticals, Inc. and the persons party thereto.	8-K	10.1	3/19/2019	001-38295
4.11	Registration Rights Agreement, dated March 18, 2021, by and among X4 Pharmaceuticals, Inc. and the persons party thereto.	8-K	10.2	3/19/2019	001-38295

4.12	Form of March 2021 Prefunded Warrant	8-K	4.1	3/19/2019	001-38295
4.13	Securities Purchase Agreement, dated November 5, 2021, by and among X4 Pharmaceuticals, Inc. and the persons party thereto.	8-K	10.1	11/5/2021	001-38295
4.14	Registration Rights Agreement, dated November 5, 2021, by and among X4 Pharmaceuticals, Inc. and the persons party thereto.	8-K	10.2	11/5/2021	001-38295
4.15	Form of November 2021 Prefunded Warrant	8-K	4.1	11/5/2021	001-38295
4.16	Controlled Equity Offering℠ Sales Agreement, dated as of August 7, 2020, by and between X4 Pharmaceuticals, Inc. and B. Riley Securities, Inc., Cantor Fitzgerald & Co. and Stifel, Nicolaus & Company, Incorporated.	S-3	1.2	8/7/2020	001-38295
4.17	Description of Registered Securities	10-K	4.17	3/21/2023	001-38295
4.18	Form of March 2022 Prefunded Warrant	8-K	4.1	3/3/2022	001-38295
4.19	Securities Purchase Agreement, dated March 3, 2022, by and among X4 Pharmaceuticals, Inc. and the persons party thereto.	8-K	10.1	3/3/2022	001-38295
4.20	Registration Rights Agreement, dated March 3, 2022, by and among X4 Pharmaceuticals, Inc. and the persons party thereto.	8-K	10.2	3/3/2022	001-38295
4.21	Purchase Agreement, dated as of January 14, 2022, by and between X4 Pharmaceuticals, Inc. and Lincoln Park Capital Fund, LLC.	8-K	10.1	1/14/2022	001-38295
4.22	Registration Rights Agreement, dated as of January 14, 2022, by and between X4 Pharmaceuticals, Inc. and Lincoln Park Capital Fund, LLC.	8-K	10.2	1/14/2022	001-38295
4.23	Securities Purchase Agreement, dated June 30, 2022, by and among X4 Pharmaceuticals Inc. and the persons party thereto.	8-K	10.1	7/1/2022	001-38295
4.24	Registration Rights Agreement, dated June 30, 2022, by and among X4 Pharmaceuticals Inc. and the persons party thereto.	8-K	10.2	7/1/2022	001-38295
4.25	Registration Rights Agreement, dated May 15, 2023, by and among X4 Pharmaceuticals, Inc. and the persons party thereto.	8-K	10.2	5/16/2023	001-38295
4.26	Securities Purchase Agreement, dated May 15, 2023, by and among X4 Pharmaceuticals, Inc. and the persons party thereto.	8-K	10.1	5/16/2023	001-38295
4.27	Form of July 2022 Pre-Funded Warrant	8-K	4.1	7/1/2022	001-38295
4.28	Form of July 2022 Warrant	8-K	4.2	7/1/2022	001-38295
4.29	Form of December 2022 Pre-Funded Warrant	8-K	4.1	12/9/2022	001-38295
4.30	Form of Class C Warrant.	8-K	4.2	12/9/2022	001-38295
4.31	Form of May 2023 Pre-Funded Warrant.	8-K	4.1	5/16/2023	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@	Amended and Restated 2017 Equity Incentive Plan	S-8	99.1	6/10/2020	333-239082
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@	Form of Performance-Based Restricted Stock Unit	S-8	99.6	6/10/2020	333-239082
10.10@	X4 Pharmaceuticals Inc. Amended and Restated 2017 Employee Stock Purchase Plan	10-Q	10.4	8/10/2023	001-38295
10.11@	X4 Pharmaceuticals, Inc. 2019 Amended and Restated Inducement Equity Incentive Plan	10-Q	10.3	8/10/2023	001-38295
10.12@	Form of Stock Option Agreement under the 2019 Inducement Equity Incentive Plan	8-K	10.2	6/17/2019	001-38295
10.13@	Form of Restricted Stock Agreement under the 2019 Inducement Equity Incentive Plan	8-K	10.3	6/17/2019	001-38295
10.14@	Form of Restricted Stock Unit Agreement under the 2019 Inducement Equity Incentive Plan	8-K	10.4	6/17/2019	001-38295
10.15@	Form of Indemnification Agreement (for directors and executive officers)	S-1/A	10.36	11/06/2017	001-38295
10.16@	Director Compensation Policy	10.16	10.16	3/21/2023	001-38295
10.17@	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
10.18@	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-Q	10.1	3/31/2020	001-38295
10.19@	Second Amended and Restated Executive Employment Agreement, dated as of March 7, 2022 by and between the Company and Adam S. Mostafa.	10-K	10.19	3/17/2022	001-38295
10.20@	Amended and Restated Executive Employment Agreement, dated as of March 7, 2022 by and between the Company and Mary DiBiase	10-K	10.23	3/17/2022	001-38295
10.21@	Executive Employment Agreement, dated as of November 14, 2022, by and between the Company and Murray Stewart	10-K	10.21	3/21/2023	001-38295
10.22#	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
10.23#	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
10.24#	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
10.25#	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
10.26	License Agreement, dated as of November 13, 2020, by and between X4 Pharmaceuticals Inc. and the Dana Farber Cancer Institute.	10-K	10.33	3/19/2021	001-38295
10.27+	Second Amended and Restated Loan and Security Agreement, dated as of January 6, 2023, by and among X4 Pharmaceuticals, Inc., X4 Therapeutics, Inc., Hercules Capital, Inc. and Hercules Capital Funding IV LLC and Hercules Capital Funding Trust 2022-1	10-K	10.27	3/21/2023	001-38295
10.28	Lease, dated as of November 11, 2019, by and between X4 Pharmaceuticals Inc. and Beacon North Village, LLC.	10-K	10.32	3/12/2020	001-38295
10.29	Master Services Agreement, dated September 10, 2015, by and between X4 Pharmaceuticals Inc. and Mayne Pharma Inc. (formerly known as Metrics, Inc.).	10-K	10.35	3/12/2020	001-38295

10.30	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-K	10.36	3/12/2020	001-38295
10.31	Amendment No. 2 to Master Services Agreement, dated February 28, 2020, by and between X4 Pharmaceuticals Inc. and Mayne Pharma Inc.	10-K	10.37	3/12/2020	001-38295
10.32	Master Services Agreement, dated February 19, 2016, by and between X4 Pharmaceuticals Inc. and Aptuit (Oxford) Limited	10-K	10.38	3/12/2020	001-38295
10.33	Amendment No. 1 to Master Services Agreement, dated February 19, 2016, by and between X4 Pharmaceuticals Inc. and Aptuit (Oxford) Limited	10-K	10.39	3/12/2020	001-38295
10.34	Amendment No. 2 to Master Services Agreement, dated February 19, 2021, by and between X4 Pharmaceuticals Inc. and Aptuit (Oxford) Limited.	10-K	10.48	3/19/2021	001-38295
10.35@	Form of Stock Appreciation Right Agreement under the X4 Pharmaceuticals, Inc. Amended and Restated 2017 Equity Incentive Plan.	8-K	10.1	11/9/2022	001-38295
10.36+	First Amendment to Second Amended and Restated Loan and Security Agreement, dated as of August 2, 2023, by and among X4 Pharmaceuticals, Inc., X4 Therapeutics, Inc, Hercules Capital, Inc. and Hercules Capital Funding IV LLC and Hercules Capital Funding Trust 2022-1.	10-Q	10.1	11/09/2023	001-38295
10.37*	Amendment No. 3 to Master Services Agreement, dated August 3, 2023, by and between X4 Pharmaceuticals Inc., and Catalent.				
21.1	List of Subsidiaries	10-K	21.1	3/17/2022	001-38295
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 and Chief Financial Officer, as required by Section 906 of the Sarbanes-Oxley Act of 2002				
97.1@*	Incentive Compensation Recoupment Policy				
101.INS	Inline XBRL Instance Document				
101.SCH	Inline XBRL Taxonomy Extension Schema Document				
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document				
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document				
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document				
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document				
104	Cover Page Interactive Data File (formatted as Inline XBRL)				

* Filed herewith

** The certifications furnished in Exhibit 32.1 hereto is deemed to accompany this Annual Report on Form 10-K and will not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended. Such certifications will not be deemed to be incorporated by reference into any filings under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, except to the extent that the Registrant specifically incorporates it by reference.

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) is the type of information that the Registrant treats as private or confidential.

+ Certain schedules and exhibits have been omitted from this Exhibit pursuant to Item 601(a)(5) of Regulation S-K. The Registrant will furnish a copy of any omitted schedule or exhibit to the U.S. Securities and Exchange Commission or its staff upon request.

@ Indicates management contract or compensatory plan

The agreements and other documents filed as exhibits to this Annual Report on Form 10-K are not intended to provide factual information or other disclosure other than with respect to the terms of the agreements or other documents themselves, and you should not rely on them for that purpose. In particular, any representations and warranties made by us in these agreements or other documents were made solely within the specific context of the relevant agreement or document and may not describe the actual state of affairs as of the date they were made or at any other time.

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 21, 2024

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 21, 2024
/s/ Adam S. Mostafa Adam S. Mostafa	Chief Financial Officer and Treasurer *(Principal Financial Officer and Principal Accounting Officer)*	March 21, 2024
/s/ Michael S. Wyzga Michael S. Wyzga	Chairman of the Board of Directors	March 21, 2024
/s/ William Aliski William E. Aliski	Director	March 21, 2024
/s/ Gary J. Bridger Gary J. Bridger, Ph.D.	Director	March 21, 2024
/s/ Francoise De Craecker Francoise De Craecker	Director	March 21, 2024
/s/ Alison Lawton Alison F. Lawton	Director	March 21, 2024
/s/ David McGirr David McGirr	Director	March 21, 2024
/s/ Murray W. Stewart Murray W. Stewart, M.D.	Director	March 21, 2024
/s/ Robert K. Woods Robert K. Woods	Director	March 21, 2024

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, 2023 and 2022, and the related consolidated statements of operations and comprehensive loss, of redeemable common stock and stockholders' equity and of cash flows for each of the three years in the period ended December 31, 2023, 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, 2023 and 2022, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2023 in conformity with accounting principles generally accepted in the United States of America.

Substantial Doubt about the Company's Ability to Continue as a Going Concern

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 1 to the consolidated financial statements, the Company has incurred operating losses and negative cash flows from operations since inception, that raise substantial doubt about its ability to continue as a going concern. Management's plans in regard to these matters are also described in Note 1. The consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty.

Basis for Opinion

These 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 audit 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.

Critical Audit Matters

The critical audit matter communicated below is a matter arising from the current period audit of the consolidated financial statements that was communicated or required to be communicated to the audit committee and that (i) relates to accounts or disclosures that are material to the consolidated financial statements and (ii) involved our especially challenging, subjective, or complex judgments. The communication of critical audit matters does not alter in any way our opinion on the consolidated financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which it relates.

External Research and Development Costs

As described in Note 2 to the consolidated financial statements, costs associated with internal research and development and external research and development services, including drug development and preclinical studies, are expensed as incurred. The Company's research and development expense for the year ended December 31, 2023 was $72.0 million, a portion of which relates to external research and development costs. Management 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. As disclosed by management, this process involves reviewing open contracts and purchase orders, communicating with applicable personnel to identify services that have been performed, and estimating the level of service performed and the associated cost incurred for the service when the Company has not yet been invoiced or otherwise notified of actual costs.

The principal consideration for our determination that performing procedures relating to external research and development costs is a critical audit matter is a high degree of auditor effort in performing procedures related to the Company's external research and development costs.

Addressing the matter involved performing procedures and evaluating audit evidence in connection with forming our overall opinion on the consolidated financial statements. These procedures included, among others, testing external research and development costs on a sample basis, which included tracing relevant information to the underlying contract research organization and contract manufacturing organization agreements, purchase orders, invoices received, and information received from certain third party service providers, where applicable.

/s/ PricewaterhouseCoopers LLP

Boston, Massachusetts

March 21, 2024

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, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 99,216	$ 121,718
Marketable securities	15,000	—
Research and development incentive receivable	562	1,152
Prepaid expenses and other current assets	7,298	5,807
Total current assets	122,076	128,677
Property and equipment, net	745	1,104
Goodwill	17,351	17,351
Right-of-use assets	5,650	7,229
Other assets	1,436	1,225
Total assets	$ 147,258	$ 155,586
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 8,947	$ 7,777
Accrued expenses	12,816	12,034
Current portion of lease liability	1,099	1,198
Current portion of long-term debt	—	1,315
Total current liabilities	22,862	22,324
Long-term debt, including accretion, net of discount	54,570	32,304
Lease liabilities	2,612	3,603
Warrant liability (Note 4)	15,683	23,131
Other liabilities	432	173
Total liabilities	96,159	81,535
Commitments and contingencies (Note 9)		
Stockholders' equity:		
Common stock, $0.001 par value. 500,000,000 shares authorized as of December 31, 2023 and December 31, 2022, respectively; 167,434,595 and 121,667,250 shares issued and outstanding as of December 31, 2023 and December 31, 2022, respectively	167	122
Additional paid-in capital	528,956	450,786
Accumulated other comprehensive loss	(119)	(119)
Accumulated deficit	(477,905)	(376,738)
Total stockholders' equity	51,099	74,051
Total liabilities and stockholders' equity	$ 147,258	$ 155,586

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 share and per share amounts)

	Year Ended December 31,		
	2023	2022	2021
Operating expenses:			
Research and development	72,017	61,058	50,647
Selling, general and administrative	35,505	27,020	24,702
Goodwill impairment	—	—	9,758
Gain on sale of nonfinancial assets	—	(509)	—
Total operating expenses	107,522	87,569	85,107
Loss from operations	(107,522)	(87,569)	(85,107)
Other income (expense):			
Interest income	4,582	219	10
Interest expense	(5,777)	(3,993)	(3,642)
Change in fair value of warrant and derivative liabilities	7,074	1,701	(366)
Other income (expense)	554	(4,197)	426
Total other income (expense), net	6,433	(6,270)	(3,572)
Loss before provision for income taxes	(101,089)	(93,839)	(88,679)
Provision for income taxes	78	28	17
Net loss and comprehensive loss	(101,167)	(93,867)	(88,696)
Deemed dividend on Class B Warrant price reset	—	(2,546)	(13,943)
Net loss attributable to common stockholders	$ (101,167)	$ (96,413)	$ (102,639)
Net loss per share attributable to common stockholders—basic and diluted	$ (0.57)	$ (1.52)	$ (3.99)
Weighted average shares of common stock outstanding—basic and diluted	177,812,480	63,525,845	25,748,797

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

X4 PHARMACEUTICALS, INC.

CONSOLIDATED STATEMENTS OF REDEEMABLE COMMON STOCK AND STOCKHOLDERS' EQUITY

(In thousands, except share amounts)

	Redeemable Common Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount	Shares	Amount				
Balance at December 31, 2020	—	—	16,305,731	$ 16	$ 267,077	$ (119)	$ (194,175)	**$ 72,799**
Issuance of common stock, redeemable common stock, and pre-funded warrants for the purchase of common stock, net of issuance costs of $4.4 million	229,885	1,875	9,435,951	10	73,858			**73,868**
Issuance of common stock under employee stock purchase plan			45,816		219			**219**
Exercise of stock options			5,860		40			**40**
Exercise of pre-funded warrants			2,129,768	2				**2**
Vesting of restricted stock units			204,531					—
Repurchase and retirement of redeemable common stock	(229,885)	(1,875)						—
Stock-based compensation					6,180			**6,180**
Net loss							(88,696)	**(88,696)**
Balance at December 31, 2021	—	—	28,127,657	$ 28	$ 347,374	$ (119)	$ (282,871)	**$ 64,412**
Issuance of common stock, warrants and prefunded warrants for the purchase of common stock, net of issuance costs of $4.7 million			92,461,988	92	59,270			**59,362**
Vesting of restricted stock units, less shares withheld and retired to satisfy tax obligations			372,831	—	(13)			**(13)**
Exercise of warrants and prefunded warrants			499,871	1				**1**
Stock-based compensation expense					5,199			**5,199**
Issuance of shares of common stock under employee stock purchase plan			204,903	1	202			**203**
Reclassification of warrant liability to equity (Note 10)					38,754			**38,754**
Net loss							(93,867)	**(93,867)**
Balance at December 31, 2022	—	—	121,667,250	$ 122	$ 450,786	$ (119)	$ (376,738)	**$ 74,051**
Issuance of common stock, warrants and prefunded warrants for the purchase of common stock, net of issuance costs of $4.6 million			34,521,046	35	60,408			**60,443**
Vesting of restricted stock units, less shares withheld and retired to satisfy tax obligations			3,510,491	2	(2)			—
Exercise of stock options and warrants			7,477,845	8	8,805			**8,813**
Stock-based compensation expense					8,687			**8,687**
Issuance of shares of common stock under employee stock purchase plan			257,963		272			**272**
Net loss							(101,167)	**(101,167)**
Balance at December 31, 2023	—	—	**167,434,595**	**$ 167**	**$ 528,956**	**$ (119)**	**$ (477,905)**	**$ 51,099**

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

X4 PHARMACEUTICALS, INC.

CONSOLIDATED STATEMENTS OF CASH FLOWS

(In thousands)

	Year Ended December 31,		
	2023	**2022**	**2021**
Cash flows from operating activities:			
Net loss	$ (101,167)	$ (93,867)	$ (88,696)
Adjustments to reconcile net loss to net cash used in operating activities:			
Stock-based compensation expense	8,687	5,199	6,180
Depreciation and amortization expense	419	513	499
Goodwill impairment	—	—	9,758
Non-cash lease expense	1,577	1,481	1,393
Accretion of debt discount	929	918	756
Change in fair value of warrant and derivative liability	(7,074)	2,881	366
Other	(227)	37	337
Changes in operating assets and liabilities:			
Prepaid expenses, other current assets and research and development incentive receivable	(1,370)	(610)	(1,755)
Accounts payable	1,234	3,425	1,166
Accrued expenses and other long-term liabilities	1,608	3,803	(152)
Operating lease liabilities	(1,128)	(882)	(698)
Operating lease right-of-use asset, net of non-cash portion	—	—	(59)
Net cash used in operating activities	(96,512)	(77,102)	(70,905)
Cash flows from investing activities:			
Purchase of property, equipment and intangible assets	(60)	(103)	(615)
Purchase of marketable securities	(16,823)	—	—
Sales and maturities of marketable securities	2,000	—	—
Net cash used in investing activities	(14,883)	(103)	(615)
Cash flows from financing activities:			
Proceeds from exercise of stock options, warrants and pre-funded warrants and issuance of shares of common stock under employee stock purchase plans	8,712	208	260
Issuance costs for amendments to loan and security agreement and for the sale of warrants accounted for as a liability (Note 10)	(631)	(4,802)	—
Employee taxes paid related to net share settlement of vested restricted stock units	—	(12)	—
Proceeds from borrowings under loan and security agreements	22,500	—	—
Repayments of borrowings under loan and security agreement	(2,064)	(795)	—
Proceeds from sale of shares of common stock, redeemable common stock, warrants and pre-funded warrants, net of issuance costs	59,999	122,631	75,985
Settlement and retirement of redeemable common stock	—	—	(2,000)
Net cash provided by financing activities	88,516	117,230	74,245
Effect of exchange rate changes on cash, cash equivalents and restricted cash	99	(105)	(319)
Net (decrease) increase in cash, cash equivalents and restricted cash	(22,780)	39,920	2,406
Cash, cash equivalents and restricted cash at beginning of period	123,028	83,108	80,702
Cash, cash equivalents and restricted cash at end of period	$ 100,248	$ 123,028	$ 83,108
Supplemental disclosure of cash flow information			
Cash paid for interest	$ 4,604	$ 3,006	$ 2,906
Supplemental disclosure of non-cash investing and financing activities:			
Acquisition of right-of-use assets financed by lease liabilities	$ —	$ —	$ 1,343
Issuance costs not yet paid	$ —	$ 661	$ 24

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

X4 PHARMACEUTICALS, INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(Unaudited)

1. NATURE OF THE BUSINESS AND BASIS OF PRESENTATION

X4 Pharmaceuticals, Inc. (together with its subsidiaries, the "Company") is a late-stage clinical biopharmaceutical company discovering and developing novel therapeutics for the treatment of rare diseases and those with limited treatment options, with a focus on conditions resulting from dysfunction of the immune system. The Company's lead clinical candidate is mavorixafor, a small-molecule antagonist of the chemokine receptor CXCR4 that is being developed as an oral, once-daily therapy. Due to its ability to increase the mobilization of mature, functional white blood cells from the bone marrow into the bloodstream, the Company believes that mavorixafor has the potential to provide therapeutic benefit across a variety of chronic neutropenic disorders, including WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) syndrome ("WHIM"), a rare, primary immunodeficiency. Following announcement of positive top-line data from the Company's pivotal, global, Phase 3 clinical trial in November 2022, the Company submitted a New Drug Application ("NDA") to the U.S. Food and Drug Administration ("FDA") in August 2023, seeking approval of oral, once-daily mavorixafor in the treatment of people aged 12 years and older with WHIM syndrome. The FDA accepted the NDA on October 30, 2023 for Priority Review, establishing a Prescription Drug User Fee Act ("PDUFA") action date of April 30, 2024. As the Company prepares for a potential launch of mavorixafor for WHIM in the U.S. in the second quarter of 2024, the Company is also enrolling participants in a Phase 2 clinical trial evaluating the safety and efficacy of mavorixafor in people with certain chronic neutropenic disorders. The Company also expects to initiate a pivotal, global, Phase 3 clinical trial in the first half of 2024 in certain chronic neutropenic disorders. The Company is headquartered in Boston, Massachusetts and has a research facility in Vienna, Austria.

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)* ("ASU 2014-15"), 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. The Company does not yet have an approved drug product. Since inception, the Company has incurred significant operating losses and negative cash flows from operations. As of December 31, 2023, the Company had $114.2 million of cash, cash equivalents and short-term marketable securities and an accumulated deficit of $477.9 million. For the year ended December 31, 2023, the Company's net losses were $101.2 million and net cash used in operating activities of $96.5 million. The Company has a covenant under its Second Amended and Restated Loan and Security Agreement (the "Hercules Loan Agreement") with Hercules Capital Inc. ("Hercules"), as most recently amended in August 2023, that requires that the Company currently maintain a minimum level of cash of $20 million, subject to adjustments beginning January 31, 2025. Based on its cash flow projections, as discussed below, the Company believes it would not maintain the minimum cash required to satisfy this covenant noted above beginning in the first quarter of 2025. In such event, the lender could require the repayment of all outstanding debt. Accordingly, management has concluded that the Company's accumulated deficit, history of losses, future expected losses and negative cash flows met the ASC 205-40 standard for raising substantial doubt about the Company's ability to continue as a going concern. The Company does not have adequate financial resources to fund its forecasted operating costs for at least one year after the date that these consolidated financial statements are issued. The accompanying consolidated financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might result from the outcome of this uncertainty. Accordingly, the consolidated financial statements have been prepared on a basis that assumes the Company will continue as a going concern and which contemplates the realization of assets and satisfaction of liabilities and commitments in the ordinary course of business.

The Company's cash flow projections exclude any gross profits related to the potential sale of the Company's drug upon potential FDA approval of the Company's NDA and the sale of any priority review voucher that may be received upon such approval of the Company's NDA. Projected cash flows also exclude additional borrowings under the Hercules Loan Agreement, which would become available upon FDA approval of the Company's NDA and exclude all other sources of external financing. To finance its operations in 2025 and beyond, the Company will need to raise additional capital, which cannot be assured. Unless and until the Company reaches profitability in the future, it will require additional capital to fund its operations, which could be raised through a combination of equity offerings, debt financings, other third-party funding, marketing and distribution arrangements and other collaborations and strategic alliances. If the Company is unable to obtain funding, it could be forced to delay, reduce or eliminate some or all of its research and development programs, product portfolio expansion or commercialization efforts, which would adversely affect its business prospects, or it may be unable to continue operations.

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

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Use of Estimates— The preparation of the Company's consolidated financial statements in conformity with U.S. Generally Accepted Accounting Principles ("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 and the impairment or lack of impairment of long-lived assets including operating lease right-of-use assets and goodwill. 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. As of the date of issuance of these consolidated financial statements, the Company is not aware of any specific event or circumstance that would require the Company to update its estimates, assumptions and judgments or revise the carrying value of its assets or liabilities. Actual results could differ from those estimates, and any such differences may be material to the Company's consolidated financial statements.

Foreign Currency and Currency Translation— The functional currency of the Company's foreign subsidiary, X4 Austria, is the U.S. dollar. However, X4 Austria maintains its books and records in Euro. As a result, monetary assets and liabilities are translated at current exchange rates as of the balance sheet date, non-monetary assets such as property and equipment and equity accounts are translated at historic rates and income and expenses are translated at the average exchanges rates for the period. Adjustments resulting from the translation of the consolidated financial statements of X4 Austria into U.S. dollars are included in the determination of net loss and are recorded in other expense, net.

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, marketable securities 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's marketable securities and cash equivalents are invested in high quality, U.S. government obligations such as U.S. Treasury bills and U.S. government agency obligations. The Company has not experienced losses related to its cash and cash equivalents.

The Company is dependent on third-party manufacturers to supply its drug substance and clinical drug supply for research and development activities in its programs. Should the Company receive FDA approval of its NDA, these third-party manufacturers would also supply the Company's drug product. 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 90 days or less at the date of purchase to be cash equivalents. Cash equivalents consisted of money market funds, treasury bills and federal government agency notes as of December 31, 2023 and December 31, 2022.

Marketable Securities— Marketable securities consist of short-term debt securities classified as available-for-sale having maturities greater than 90 days, but less than 365 days from the date of acquisition (settlement). The Company determines the appropriate classification of the securities at the time they are acquired and evaluate the appropriateness of such classifications at each balance sheet date. The Company's marketable securities, which consist of U.S. Treasury securities and federal government agency notes, are classified as available-for-sale securities whose fair value is categorized as Level 2 as their value is based on valuations using significant inputs derived from, or corroborated by, observable market data. The cost of available-for-sale securities sold is based on the specific-identification method. Unrealized gain and losses on available-for-sale are included as a component of other comprehensive loss on the consolidated balance sheet and as a component of total comprehensive loss on the consolidated statement of operations and comprehensive loss until realized. Realized gains and losses on the sale of marketable securities are determined using the specific-identification method and recorded in other (expense) income, net on the

accompanying condensed consolidated statements of operations and comprehensive loss. The Company reviews marketable securities for impairment whenever the fair value of a marketable security is less than the amortized cost and evidence indicates that a marketable security's carrying amount is not recoverable. Unrealized losses are evaluated for impairment under ASC 326, *Financial Instruments - Credit Losses*, to determine if the impairment is credit-related or noncredit-related. Credit-related impairment is recognized as an allowance on the consolidated balance sheets with a corresponding adjustment to earnings, and noncredit-related impairment is recognized in other comprehensive loss, net of taxes. Evidence considered in this assessment includes reasons for the impairment, compliance with our investment policy, the severity of the impairment, collectability of the security, and any adverse conditions specifically related to the security, an industry, or geographic area.

Restricted Cash

(in thousands)	As of December 31, 2023	As of December 31, 2022
Letter of credit security: Waltham lease	$ 250	$ 250
Letter of credit security: Vienna Austria lease	211	205
Letter of credit security: Boston lease	571	855
Total restricted cash	$ 1,032	$ 1,310
Restricted cash included in prepaid expenses and other current assets	$ 250	$ 285
Restricted cash included in other assets	$ 782	$ 1,025

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 respective landlord. The Company's Waltham lease agreement expired in December 2023; however, the letter of credit will remain in place until the landlord completes its lease expiration procedures. In accordance with the Company's Hercules Loan Agreement and as further described in Note 7, the Company at all times must maintain a minimum level of cash of $20.0 million in an account or accounts in which Hercules has a first priority security interest as further described in Note 7.

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 statements of cash flows as of December 31, 2023, 2022 and 2021:

(in thousands)	December 31, 2023	December 31, 2022	December 31, 2021
Cash and cash equivalents	$ 99,216	$ 121,718	$ 81,787
Restricted cash, current (included within prepaid expenses and other current assets)	250	285	—
Restricted cash, non-current (included within other assets)	782	1,025	1,321
Total cash, cash equivalents and restricted cash	$ 100,248	$ 123,028	$ 83,108

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 to 7 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— The Company accounts for leases in accordance with Accounting Standards Codification ("ASC"), Topic 842, *Leases* ("ASC 842"). Under ASC 842, 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 referenced the effective rate of its Hercules Loan Agreement, as adjusted for differences terms, to determine its incremental borrowing rate for each of its operating leases at lease inception.

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 office and laboratory leases beginning in 2019 and after, the Company has elected to account for the lease and non-lease components as a combined lease component.

Impairment of Long-Lived Assets— Long-lived assets consist of property and equipment and operating lease right-of-use assets. 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, a significant decline in the price of the Company's common stock, 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. See Note 4 for more information on the Company's goodwill impairment tests as of December 31, 2023, 2022 and 2021.

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.

The embedded derivative liability related to the redemption features of the Company's debt with Hercules as described further below and the Company's outstanding Class C warrants are carried at fair value and using a Level 3 measurements. The Company's cash equivalents, which consist of money market funds, which are invested in U.S. Treasury securities and U.S. government agency obligations, are carried at fair value, determined based on Level 1 and Level 2 inputs in the fair value hierarchy described above. The Company's marketable securities are carried at fair value determined based on Level 2 inputs. 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, 2023 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 has defined its Chief Operating Decision Maker ("CODM") as its Chief Executive Officer. The CODM manages the Company's operations as a single operating segment, which comprises its single reportable 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.

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 selling, 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, nonemployees 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 also issued stock-based awards with performance-based vesting conditions that vest in part upon the Company's achievement of operational milestones and over time thereafter for the subsequent two years as the employee continues to provide services. The Company assesses the probability of achievement of these operational milestones and recognizes stock-based compensation for these awards using the accelerated attribution model based on the fair value of the awards as of the date of grant and its best estimate of the date each operational milestone will be achieved.

The Company classifies stock-based compensation expense in its consolidated statements 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 was 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.

Derivative Liabilities: Hercules Loan Redemption Feature— The Company's loan agreement with Hercules (see Note 7) 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, or, at its election, charge additional contingent interest fees on any overdue interest or principal payments. 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.

Comprehensive Loss— For the year ended December 31, 2023, 2022, and 2021 all foreign currency remeasurement gains and losses were included in net loss as the Company has deemed the functional currency of its foreign subsidiary to be the U.S. Dollar. Accumulated other comprehensive loss includes foreign currency translation adjustments of $119 thousand for the year ended December 31, 2019 that were included in other comprehensive loss prior to the designation of the U.S. Dollar as the functional currency of X4 Austria. As of December 31, 2023, unrealized gains on the Company's marketable debt security portfolio were nominal in included within interest income on the consolidated statement of operations and comprehensive loss.

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—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. In addition, during the years ended December 31, 2022 and 2021, in accordance with the provisions of the Company's Class B Warrants, the exercise price of each outstanding Class B Warrant was adjusted to the price of subsequent sales of common stock. Such adjustment is presented as a deemed dividend that adjusts net loss available to common shareholders for purposes of basic earnings per share. The deemed dividend is calculated using the Black-Scholes pricing model, taking into account historical volatility of the Company's common stock and the estimated remaining life of the outstanding Class B Warrants.

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, unvested restricted stock units and warrants to purchase shares of common stock are considered potential dilutive shares of common stock.

Recently Adopted Accounting Standards Not Yet Adopted

In November 2023, the Financial Accounting Standards Board ("FASB") issued ASU 2023-07, *Segment Reporting (Topic 326) Improvements to Reportable Segment Disclosures* ("ASU 2023-07"). Among other disclosure enhancements, ASU 2023-07 requires that entities with one reportable segment, such as the Company, disclose general information for its reportable segment, such as the title and position of the individual identified as the CODM, which for the Company is the Chief Executive Officer, the types of products and services provided by the reportable segment, the measure of profit or loss reviewed by the CODM to evaluate performance of the reportable segment and other financial results such as interest income, interest expense and depreciation associated with the reportable segment. The amendments in ASU 2023-07 will become effective for the Company in its consolidated financial statements as of and for the three years ending December 31, 2024 and must be adopted retrospectively. Although the Company continues to evaluate the potential impact of ASU 2023-07, the Company does not believe that the adoption of ASU 2023-07 will have a material impact on its consolidated financial statement when adopted.

In December 2023, the FASB issued ASU 2023-09, *Income Taxes (Topic 740) Improvements to Income Tax Disclosures* ("ASU 2023-09"). The amendments in ASU 2023-09 require that entities on an annual basis disclose specific categories in the income tax rate reconciliation and provide additional information for reconciling items if the effect of those reconciling items that exceed a certain threshold. ASU 2023-09 will also require more disaggregated disclosures related to income taxes paid. The amendments in ASU 2023-09 will become effective for the Company in its December 31, 2024 consolidated financial statements. Although the Company continues to evaluate the impact of ASU 2023-09, the Company expects that these amendments will require further disclosures in the tax footnote of its annual consolidated financial statements and will not have a material impact on its

consolidated financial states when adopted.

3. LICENSE, COLLABORATION AND FUNDING ARRANGEMENTS

Genzyme Agreement

In July 2014, the Company entered into a license agreement with Genzyme (the "Genzyme Agreement") 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.

During 2023, a $5.0 million milestone was incurred upon the FDA's acceptance of the Company's NDA seeking approval of oral, once-daily mavorixafor in the treatment of people aged 12 years and older with WHIM syndrome. Such amount has been included within research and development expense and was paid in the fourth quarter of 2023.

As of December 31, 2023, the Company is obligated to make future milestone payments in the aggregate amount of up to $20.0 million contingent upon the achievement by the Company of certain clinical-stage regulatory and sales milestones with respect to licensed products. A $7.0 million regulatory milestone payment, which would be payable upon the FDA's approval of the Company's NDA, is reasonably possible of becoming payable with the next five to six months under the Genzyme Agreement. The remaining regulatory milestones include (i) $3.0 million for the acceptance by the EMA of the Company's first drug application and (ii) $5.0 million upon the notification by the EMA of regulatory approval of our first drug application. The Company must also make one-time sales milestone payments of $0.5 million, $1.5 million and $3.0 million on cumulative net sales of $50.0 million, $150.0 million and $300.0 million, respectively.

The Company is also obligated to pay Genzyme tiered royalties based on net sales of licensed products that the Company commercializes under the agreement. Upon the first sale of the Company's drug candidate in the U.S., the Company will incur a royalty on annual net sales at a rate of 6% up to $150 million, 10% on the portion of annual net sales between $150 million and $300 million, and 12% thereafter on annual sale over $300 million.

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.

For the years ended December 31, 2022 and 2021, 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, 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 its product. 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 years ended December 31, 2023, 2022 and 2021, 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 years ended December 31, 2023, 2022 and 2021.

Dana Farber Cancer Institute Agreement

In November 2020, the Company entered into a license agreement (the "DFCI Agreement") with the Dana Farber Cancer Institute ("DFCI") pursuant to which the Company obtained a non-exclusive, royalty-bearing license to use, make, have made, develop, market, import, distribute, sell and have sold products covered by patent rights owned by DFCI. Under the terms of the DFCI Agreement, the Company paid a one-time, upfront fee of $25 thousand and approximately $35 thousand for reimbursement of DFCI's past patent expenses relating to the patent rights. The Company will pay 25% of DFCI's ongoing patent prosecution expenses and an annual license maintenance fee of $10 thousand in each of the first three years, $40 thousand in each of the subsequent three years and $50 thousand every year after that until commercialization. The Company may be required to make milestone payments of up to an aggregate of approximately $32.3 million related to development, regulatory and commercial sales events. No such milestone payments have been incurred to date. 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. The term of the DCFI Agreement will continue until the expiration of the last valid claim within the patent rights covering the product. DFCI may terminate the agreement if certain events occur.

Research and Development Incentive Program

The Company 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 X4 Austria is 14% for the current year. X4 Austria also participated in a COVID-19 incentive program, which provides reimbursement for qualified capital spending during a defined time period.

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 the years ended December 31, 2023 and 2022, the amounts due under these programs were $0.6 million and $1.2 million, respectively, which is included in research and development incentive receivable on the consolidated balance sheets. During the years ended December 31, 2023, 2022 and 2021, the Company recorded $0.6 million, $0.5 million and $0.8 million, respectively, 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 with Abbisko (the "Abbisko Agreement"). 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, 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. 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. Assuming mavorixafor is developed by Abbisko in six indications, the Company would be entitled to milestone payments of up to $214.0 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 Territory.

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 re-evaluates 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.

4. 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, 2023 Using:			
(in thousands)	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents--money market funds and U.S. Treasury bills	$ 76,856	$ 4,985	$ —	$ 81,841
Marketable securities-U.S. Treasury notes, U.S. Treasury bills, and federal government agency notes	—	15,000	—	15,000
	$ 76,856	$ 19,985	$ —	$ 96,841
Liabilities:				
Embedded derivative liability	$ —	$ —	$ 10	$ 10
Class C Warrant Liability (Note 10)			15,683	15,683
	$ —	$ —	$ 15,693	$ 15,693

	Fair Value Measurements as of December 31, 2022 Using:			
(in thousands)	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents—money market funds	$ 70,170	$ 2,858	$ —	$ 73,028
	$ 70,170	$ 2,858	$ —	$ 73,028
Liabilities:				
Embedded derivative liability	$ —	$ —	$ 10	$ 10
Class C warrant liability			23,131	23,131
	$ —	$ —	$ 23,141	$ 23,141

All marketable securities are classified as short-term investments as all are due within one year and include investments in U.S. Treasury notes, U.S. Treasury bills and federal government agency notes. The amortized cost of each investment, individually and in aggregate, approximates fair value. The Company evaluated each marketable security for impairment that is other-than-temporary and concluded that no marketable security was impaired as of December 31, 2023.

The Company's cash equivalents consisted of money market funds invested in U.S. Treasury securities and direct investments in U.S. Treasury securities. The money market funds were valued based on quoted prices in active markets for identical assets, which represents a Level 1 measurement. U.S. Treasury securities were valued by using inputs observable in active markets for similar securities, which represents a Level 2 measurement in the fair value hierarchy.

(in thousands)	Amortized Cost	Gross Unrealized	Gross Unrealized	Fair Value
U.S. Treasury securities	2927000	3000	0	2930000
Federal Government Agency Securities	12068000	7000	5000	12070000
Total available-For-sale debt securities	14995000	10000	5000	15000000

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

(in thousands)	Embedded Derivative Liability	PIPE Warrant Liability	Class C Warrant Liability
Balance at December 31, 2020	$ 455	$ —	$ —
Change in fair value	366	—	—
Balance at December 31, 2021	821	—	—
Issuance of Class C Warrants	—	41,249	21,526
Change in fair value	(811)	(2,495)	1,605
Reclassification to permanent equity	—	(38,754)	—
Balance at December 31, 2022	10	—	23,131
Reclassification to permanent equity upon exercise			(374)
Change in fair value	—	—	(7,074)
Balance at December 31, 2023	$ 10	$ —	$ 15,683

***Valuation of Embedded Derivative Liability*—** The fair value of the embedded derivative liability recognized in connection with the Company's loan agreement with Hercules (see Note 7), which is associated with additional fees due to Hercules upon events of default, 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 embedded derivative liability, which is reported within other non-current liabilities on the consolidated balance sheets, is 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 fees or contingent interest upon an event of default and the Company's risk-adjusted discount rate of 17%. As of December 31, 2023 and December 31, 2022, the fair value of this derivative liability was $10 thousand.

***Warrant Liabilities*—**

PIPE Warrant Liability— On July 6, 2022, the Company issued warrants for the purchase of its common stock in a private placement (the " PIPE Warrants"). Upon issuance, the holder's exercise of the PIPE Warrants was conditioned on the Company increasing it authorized shares. As there were insufficient authorized shares available at the time of issuance, the PIPE Warrants were classified as a liability and measured at fair value. On September 1, 2022, upon shareholder approval of the increase to the Company's authorized shares, the PIPE Warrants met all criteria required for permanent equity accounting and, accordingly, the Company remeasured the fair value of the warrant liability through "other income (expense)" and reclassified the fair value of the warrant liability to additional paid-in capital.

Class C Warrant Liability— In December 2022, the Company issued Class C Warrants for the purchase of shares of its common stock in a public offering. The Class C Warrants are accounted for as a liability on the consolidated balance sheet and are adjusted to fair value at period end through "other income (expense)."

The Company calculated the fair value of the PIPE Warrants and the Class C Warrants using the Black-Scholes option pricing model with the following inputs:

	PIPE Warrants		Class C Warrants		
	7/6/2022 (issuance)	9/1/2022 (reclassification to permanent equity)	12/9/2022 (issuance)	December 31, 2022	December 31, 2023
Common stock price	$1.09	$1.04	$0.93	$0.99	$0.84
Risk-free interest rate	3.0 %	3.3 %	3.8 %	4.0 %	3.9 %
Expected term (in years)	5.0	4.9	5.0	4.9	3.9
Expected volatility	97.3 %	97.5 %	101.8 %	101.7 %	96.2 %
Expected dividend yield	— %	— %	— %	— %	— %

Impairment of 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. During the fourth quarter of 2021, the Company's market capitalization, measured as the price of the Company's common stock multiplied by common shares outstanding, was below the value of the Company's net assets, including goodwill. As a result of the sustained decline in the market price of the Company's common stock, the fair value of the Company's single reporting unit, determined based on Company's market capitalization on December 31, 2021, was lower than its carrying value and the Company concluded that goodwill was impaired. Accordingly, the Company recorded an impairment charge of $9.8 million to reduce the carrying amount of goodwill to $17.4 million as of December 31, 2021. The Company tested goodwill for impairment as of December 31, 2023 and 2022 concluded that goodwill was not further impaired. Should the market value of the Company's common stock decline, additional impairment charges may be recorded in the future.

The following table provides a rollforward of the Company's goodwill and accumulated impairment losses.

(in thousands)	Goodwill, Gross	Accumulated Impairment Loss	Goodwill
Goodwill at December 31, 2020	$ 27,109	$ —	$ 27,109
Impairment losses	—	(9,758)	(9,758)
Goodwill at December 31, 2021	27,109	(9,758)	17,351
Goodwill at December 31, 2022	27,109	(9,758)	17,351
Goodwill at December 31, 2023	$ 27,109	$ (9,758)	$ 17,351

5. PROPERTY AND EQUIPMENT

Property and equipment, net consisted of the following:

(in thousands)	December 31, 2023	December 31, 2022
Leasehold improvements	$ 228	$ 228
Furniture and fixtures	1,301	1,268
Computer equipment	160	173
Software	24	24
Lab equipment	651	639
	2,364	2,332
Less: Accumulated depreciation and amortization	(1,619)	(1,228)
	$ 745	$ 1,104

Depreciation and amortization expense related to property and equipment was approximately $419 thousand, $513 thousand, and $499 thousand for the years ended December 31, 2023, 2022 and 2021, respectively.

6. ACCRUED EXPENSES

Accrued expenses consisted of the following:

(in thousands)	December 31, 2023	December 31, 2022
Accrued employee compensation and benefits	$ 8,195	$ 6,592
Accrued external research and development expenses	2,804	3,906
Accrued professional fees	1,195	571
Accrued deferred financing fees	—	591
Other	622	374
	$ 12,816	$ 12,034

7. LONG-TERM DEBT

Long-term debt consisted of the following:

(in thousands)	December 31, 2023	December 31, 2022
Principal amount of long-term debt	$ 55,000	$ 32,500
Debt discount, net of accretion	(917)	(196)
Cumulative accrual of end of term payments	487	1,315
Long-term debt	54,570	33,619
Less: current portion of long-term debt	—	(1,315)
Long-term debt, net of current portion	$ 54,570	$ 32,304

Hercules Loan Agreement

In October 2018, the Company entered into a Loan and Security Agreement, which has been subsequently amendment from time to time, with Hercules Capital Inc. In January 2023, the Company entered into a Second Amended and Restated Loan and Security Agreement (the "Second A&R Hercules Loan Agreement") with Hercules Capital, Inc., as agent and lender, and Hercules Capital Funding IV LLC and Hercules Capital Funding Trust 2022-1, as lenders (collectively, "Hercules"). On August 2, 2023, Hercules and the Company entered into an amendment (the "Amendment") to the Second A&R Hercules Loan Agreement, (as amended by the Amendment, the "Hercules Loan Agreement") with Hercules. The Hercules Loan Agreement provides for a term loan facility of up to $115.0 million, under which the Company has borrowed an aggregate of $55.0 million of term loans to date representing the maximum borrowings as of December 31, 2023. The term loan facility includes:

(i) $32.5 million outstanding (the "Conversion Balance") prior to effectiveness of the most recent amendment in August 2023;

(ii) a $22.5 million term loan tranche drawn upon the closing of the Amendment;

(iii) an additional tranche of up to $20.0 million, which will be available in either one or two drawings following potential U.S. approval of mavorixafor in individuals with WHIM syndrome ("Approval") until the earlier of (A) 45 days following Approval and (B) September 30, 2024 in the case of the first drawing, and until December 15, 2024 in the case of a second drawing;

(iv) an additional tranche of $7.5 million, which will be available following achievement of a certain clinical development-related milestone through the earlier of (A) 45 days following achievement of such milestone and (B) December 15, 2024; and

(v) an additional tranche of up to $32.5 million, which will be available subject to approval by Hercules in its sole discretion.

Borrowings under the Hercules Loan Agreement accrue interest at a variable rate equal to the greater of (i) 10.15% or (ii) *The Wall Street Journal* prime rate plus 3.15%. In an event of default and until such event is no longer continuing, the interest rate applicable to borrowings would be increased by 4.0%. Borrowings are repayable in monthly interest-only payments through March 1, 2025, and in equal monthly payments of principal and accrued interest from April 1, 2025 (the "Amortization Date") until the maturity date of the loans. The Amortization Date may be extended to October 1, 2026, if Approval occurs on or prior to

September 30, 2026 and no event of default occurs. The loans mature on October 1, 2026; provided, however, that such maturity date will be extended to July 1, 2027 if the Amortization Date is extended pursuant to the foregoing sentence. 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 2% during the one-year period from January 6, 2024 to January 5, 2025 and 1% thereafter. In addition, the Hercules Loan Agreement provides for payment of end-of-term fees of $2.1 million plus 3.5% of the aggregate principal amount of loans drawn, if any, subsequent to the Amendment, payable upon the earlier of maturity or the repayment in full of all obligations under the Hercules Loan Agreement. Borrowings under the Hercules Loan Agreement are collateralized by substantially all of the Company's personal property and other assets except for its intellectual property (but including rights to payment and proceeds from the sale, licensing or disposition of the intellectual property).

Under the Hercules Loan Agreement, the Company has agreed to affirmative and negative covenants. Prior to January 31, 2025, the Company must maintain cash in an account or accounts in which Hercules has a first priority security interest ("Qualified Cash") in an aggregate amount equal to at least $20.0 million.

- On and after January 31, 2025, such amount must equal at least 20% of the aggregate principal amount of loans outstanding under the Hercules Loan Agreement.
- From and after January 31, 2025, the Company must maintain trailing six-month net product revenue of at least 55% of its forecast as approved by the Company's Board of Directors (the "Performance Covenant"). However, the Performance Covenant will be waived during any period in which:
  (i) the Company maintains Qualified Cash in an aggregate amount equal to at least 75% of loans outstanding under the Amended Loan Agreement or
  (ii) both (x) the Company maintains a Market Capitalization (as defined in the Hercules Loan Agreement) of at least $450.0 million and (y) the Company maintains Qualified Cash in an aggregate amount equal to at least 45% of loans outstanding.

The Hercules Loan Agreement also restricts 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, with certain exceptions.

The Company recognized interest expense under the Hercules Loan Agreement as follows:

(in thousands)	For the years ended		
	2023	2022	2021
Total interest expense	$ 5,777	$ 3,989	$ 3,642
Non-cash interest expense	$ 929	$ 918	$ 756

The annual effective interest rate on the Hercules Loan Agreement as of December 31, 2023 was 13.6%. There were no principal payments due or paid under the Hercules Loan Agreement during the year ended December 31, 2023. End-of-term payments of $2.1 million and $0.8 million were paid during the years ended December 31, 2023 and 2022, respectively, in accordance with the Hercules Loan Agreement.

As of December 31, 2023, future principal payments and accrued end-of-term payments due under the Hercules Loan Agreement were as follows (in thousands):

Year Ending December 31	Total
2024	—
2025	24,720
2026	30,767
Long-term debt, including end-of-term payments	$ 55,487

As of December 31, 2022, the Company had the intent and ability to refinance the short-term principal obligations of the Hercules Loan Agreement to long-term, as demonstrated by entering into the Second A&R Hercules Loan Agreement on January 6, 2023. Therefore, short-term debt on the December 31, 2022 consolidated balance sheet includes only the accrued portion of certain end-of-term payments due within one year of the consolidated balance sheet date that remained due within one year of the

consolidated balance sheet date following the January 6, 2023 refinancing.

8. LEASES

The Company has lease agreements for its facilities in Boston, Massachusetts, which is the Company's principal executive offices, and in Vienna, Austria, which is the Company's research and development center. The Company's Waltham, Massachusetts lease, which the Company had sublet to a third party, expired in December 2023. There are no restrictions or financial covenants associated with any of the lease agreements.

Boston Lease— The Company leases approximately 28,000 square feet of office space in Boston, Massachusetts ("Boston Lease"), which serves as the Company's headquarters. Base rental payments are approximately $1.1 million annually, plus certain operating expenses. The term of the Boston Lease will continue until November 2026, unless earlier terminated. The Company has the right to sublease the premises, subject to landlord consent and also has the right to renew the Boston Lease for an additional five years at the then prevailing effective market rental rate. The Company is required to maintain a security deposit in the form of a letter of credit for $0.6 million for the benefit of the landlord.

Waltham Lease— The Company leased approximately 6,000 square feet of office space in Waltham, Massachusetts ("Waltham Lease"). The Waltham Lease, as amended, commenced on January 1, 2019, and expired on December 31, 2023. The Company was subleasing the space to a third party for the duration of the lease and such sublease income is reflected on the table below. The right-of-use asset was amortized to rent expense over the five year term of the lease and was retired upon expiration of the lease.

Vienna Austria Leases— The Company has an operating lease for approximately 1,200 square meters of laboratory and office space in Vienna, Austria ("Vienna Lease"), which commenced in February 2021 for a term of seven years. The annual base rent for the Vienna Lease is approximately $288 thousand.

The components of lease expense for the three years ended December 31, 2023, 2022 and 2021 were as follows (dollar amounts in thousands):

	For the Year Ended December 31,		
Lease Cost	**2023**	**2022**	**2021**
Fixed operating lease cost	$ 2,084	$ 2,080	$ 2,087
Short-term lease costs	—	—	42
Total lease expense	$ 2,084	$ 2,080	$ 2,129
Other information			
Operating cash outflows from operating leases	$ 1,385	$ 1,354	$ 1,257
Leased assets obtained in exchange for new operating lease liabilities	$ —	$ —	$ 1,343
Weighted-average remaining lease term—operating leases	3.2 years	4.0 years	5.0 years
Weighted-average discount rate—operating leases	11.5 %	11.4 %	11.3 %
Sublease income	$ 195	$ 196	$ 196

Maturities of lease liabilities due under lease agreements that have commenced as of December 31, 2023 are as follows (in thousands):

Maturity of lease liabilities	Operating Leases
2024	$ 1,382
2025	1,411
2026	1,341
2027	288
2028 and thereafter	47
Total lease payments	4,469
Less: interest	(758)
Total operating lease liabilities as of December 31, 2023	$ 3,711

9. COMMITMENTS AND CONTINGENCIES

The Company has agreements with clinical research organizations ("CROs") pursuant to which the Company and the CROs are conducting clinical trials of mavorixafor for the treatment of WHIM syndrome and chronic neutropenia disorders. The Company may terminate these agreements by providing notice pursuant to the contractual provisions of such agreements and would incur early termination fees. The Company also has agreements with contract manufacturing organizations ("CMOs") for the production of mavorixafor for use in clinical trials and to support the validation of the CMO's manufacturing process as part of the NDA process. The Company's agreement with the CMO who produces batches of API for use in the Company's clinical drug supply contains cancellation provisions that would require the Company to pay up to the full contract value upon cancellation. As of December 31, 2023, the Company has approximately $1.5 million of such commitments in place subject to cancellation provisions.

License Agreements— See Note 3 for a summary of the Company's license agreements, which commit the Company to contingent milestone and royalty fees based on future operational events.

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, 2023 or December 31, 2022.

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.

10. COMMON STOCK AND REDEEMABLE COMMON STOCK

Common Stock— As of December 31, 2023, the Company's Certificate of Incorporation, as amended and restated, authorizes the Company to issue 500 million shares 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.

ATM Sales Agreement

On August 7, 2020, the Company entered into a Controlled Equity Offering℠ Sales Agreement (the "ATM Sales Agreement") with B. Riley Securities, Inc., Cantor Fitzgerald & Co., and Stifel, Nicolaus & Company, Incorporated (collectively the "Sales Agents"), pursuant to which the Company may offer and sell, at the Company's sole discretion through one or more of the Sales Agents, shares of its common stock having an aggregate offering price of up to $75.0 million. To date, the Company has sold approximately $14.3 million, net of offering costs, of common stock under the ATM Sales Agreement.

Q1 2021 Private Placement

On March 18, 2021, the Company entered into a securities purchase agreement with several institutional and accredited investors pursuant to which the Company agreed to issue and sell in a private placement (the "Private Placement") an aggregate of 6,271,836 shares of common stock, 229,885 shares of redeemable common stock and, to certain investors, in lieu of common stock, pre-funded warrants to purchase an aggregate of 50,000 shares of common stock at a price of $8.70 per share of common stock (or $8.69 per pre-funded warrant). The Private Placement closed on March 23, 2021 and the Company received gross proceeds of $55.0 million, before deducting offering expenses payable by the Company.

In August 2021, the investor who purchased the redeemable common stock exercised its option to sell its 229,885 shares of redeemable common stock back to the Company at the original purchase price of $8.70 per share for an aggregate of $2.0 million. The Company adjusted the carrying amount of the redeemable common stock to its redemption value and subsequently retired these shares.

Lincoln Park Capital Fund Purchase Agreement

On January 14, 2022, the Company and Lincoln Park Capital Fund, LLC ("Lincoln Park") entered into a securities purchase agreement (the "LPC Purchase Agreement") and a registration rights agreement (the "Registration Rights Agreement"), pursuant to which the Company has the right to sell shares of common stock to Lincoln Park, having an aggregate value of up to $50.0 million, subject to certain limitations and conditions set forth in the LPC Purchase Agreement, at the Company's request from time to time during the 36-month term of the LPC Purchase Agreement. In consideration for entering into the LPC Purchase Agreement, the Company issued 230,414 shares of common stock to Lincoln Park as an initial commitment fee. Upon execution of the LPC Purchase Agreement and the Registration Rights Agreement on January 14, 2022, the Company sold to Lincoln Park, as an initial purchase under the LPC Purchase Agreement, a total of 1,382,488 shares of common stock, at a per share price of $2.17 per share, for aggregate consideration of approximately $3.0 million.

Q1 2022 PIPE

On March 3, 2022, the Company entered into a securities purchase agreement pursuant to which it agreed to issue and sell to an investor, in a private placement (the "Q1 2022 PIPE"), 900,000 shares of common stock at a price of $1.80 per share, which represents the volume weighted average price per share of the Company's common stock as quoted on the Nasdaq Stock Market for the thirty (30) consecutive-day trading day period ending on March 2, 2022, and pre-funded warrants to purchase 766,666 shares of common stock at a purchase price of $1.79 per pre-funded warrant (representing the price of $1.80 per share minus the $0.01 per share exercise price of each such prefunded warrant). The pre-funded warrants are exercisable at any time after their original issuance date and will have no expiration date. The Q1 2022 PIPE closed on March 7, 2022 and the Company received gross proceeds of $3.0 million, before deducting offering expenses payable by the Company. In accordance with an associated registration rights agreement, the Company filed a registration statement covering the resale of these securities in April 2022.

Q2 2022 PIPE

On June 30, 2022, the Company entered into a securities purchase agreement with several institutional and accredited investors pursuant to which the Company agreed to issue in a private placement (the "Q2 2022 PIPE") an aggregate of 37,649,086 shares of common stock and, to certain investors, in lieu of common stock, pre-funded warrants to purchase an aggregate of 13,276,279 shares of common stock at a price of $1.095 per share of common stock (or $1.094 per pre-funded warrant) and 50,925,365 warrants (the "Warrants") for the purchase of shares of common stock. Each Warrant has an exercise price equal to $1.095 per share and will expire on the date that is 60 months from their original issue date. The price per pre-funded warrant represents the price of $1.095 per share sold in the Q2 2022 PIPE, minus the $0.001 per share exercise price of each such pre-funded warrant. The pre-funded warrants are exercisable, subject to certain beneficial ownership restrictions, at any time after their original issuance and will not expire. The Q2 2022 PIPE closed on July 6, 2022 and the Company received gross proceeds of $55.7 million, before deducting offering expenses paid by the Company.

The exercise of any Warrant was conditioned upon the Company increasing its authorized shares. Accordingly, the Company convened a special meeting of its stockholders on September 1, 2022, during which the stockholders approved an increase in the number of authorized shares of common stock from 125 million to 500 million pursuant to an amendment to the Company's Certificate of Incorporation. As of July 6, 2022, due to the shortfall in authorized and available common shares, the Warrants did not meet the criteria required for permanent equity accounting. As a result, the Company allocated $41.2 million of the gross proceeds from the offering to the fair value of the Warrants, which was recorded as a warrant liability, and the remaining $13.5 million was allocated to the common shares and pre-funded warrants and recorded as permanent equity. The fair value of the warrant liability was calculated using the Black-Scholes option valuation model. The Company also allocated a portion of the transaction fees, including commissions and legal fees, to the warrant liability and expensed within other expense, net, approximately $2.9 million of these fees upon the closing of the Q2 2022 PIPE. Upon shareholder approval of the increase to the Company's authorized shares, the Warrants met all criteria required for permanent equity accounting and, accordingly, the Company remeasured the fair value of the warrant liability through earnings, which resulted in approximately $2.5 million of income included within other income (expense) and reclassified the fair value of the warrant liability to additional paid-in capital.

Also on June 30, 2022, the Company entered into a registration rights agreement, pursuant to which the Company agreed to register for resale the common shares issued in the Q2 2022 PIPE and the issuance of the shares of common stock underlying the Pre-Funded Warrants and the Warrants sold in the offering. Such registration statement was filed on July 29, 2022 and was declared effective by the SEC on August 5, 2022.

Q4 2022 Public Offering

On December 7, 2022, the Company sold 52,300,000 shares of common stock and, in lieu of common stock, prefunded warrants to purchase 6,800,000 shares of common stock, and accompanying Class C warrants to purchase 32,762,947 shares of its common stock. The common stock was issued at a price to the public of $1.10 per share and the accompanying Class C warrants and prefunded warrants were issued at a price of $1.099 per prefunded warrant and accompanying Class C warrant. The Class C warrants have an exercise price of $1.50, 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 December 9, 2022, were $65.1 million before deducting underwriting discounts and offering expenses.

The Company concluded that the Class C warrants do not meet the equity contract scope exception under ASC 815-40 as in the event of a fundamental transaction such as a merger certain provisions may require the Company to adjust the settlement value that is not consistent with a fixed-for-fixed option pricing model. As a result, as of issuance date, the Company allocated $21.5 million of the gross proceeds from the offering to the Class C Warrants based on their fair value, which was recorded as a warrant liability, and the remaining $43.6 million was allocated to the common shares and pre-funded warrants and recorded as permanent equity. The Class C warrant liability has been subsequently adjusted to fair value at December 31, 2022 and will be adjusted to fair value at each subsequent balance sheet date until the warrants are settled. Changes in fair value of the Class C warrants are recognized as a component of other income (expense), net in the consolidated statement of operations and comprehensive loss.

The fair value of the Class C warrant liability is calculated using the Black-Scholes option valuation model as further described in Note 4. The Company also allocated a portion of the transaction fees, including commissions and legal fees, to the Class C Warrant liability and expensed within other expense, net, approximately $1.7 million of these fees upon the closing of the Q4 2022 Public Offering.

Q2 2023 PIPE

On May 15, 2023, the Company entered into a securities purchase agreement pursuant to which it agreed to issue and sell to several institutional and accredited investors (the "Investors"), in a private placement (the "Q2 2023 PIPE"), 34,521,046 shares of common stock at a price of $1.52 per share and pre-funded warrants to purchase 8,263,157 shares of common stock at a purchase price of $1.519 per pre-funded warrant (representing the price of $1.52 per share minus the $0.001 per share exercise price of each such prefunded warrant). The pre-funded warrants are exercisable, subject to certain beneficial ownership restrictions, at any time after their original issuance and will not expire. The Q2 2023 PIPE closed on May 18, 2023. The Company received gross proceeds of $65.0 million, before deducting offering expenses paid by the Company.

Also on May 15, 2023, the Company entered into a registration rights agreement with the Investors, pursuant to which the Company agreed to register for resale the common shares issued in the Q2 2023 PIPE and the issuance of the shares of common stock underlying the pre-funded warrants held by the Investors. Such registration statement was filed on June 9, 2023 and was declared effective by the SEC on June 20, 2023.

11. COMMON STOCK WARRANTS

Class A Warrants
In connection with its issuance of common stock in public offerings that closed on April 16, 2019, the Company issued 3,900,000 Class A warrants, which are exercisable for shares of the Company's common stock or prefunded warrants to purchase shares of the Company's common stock. The Class A warrants have an exercise price of $13.20 per warrant, expire on April 15, 2024 and were immediately exercisable upon issuance.

Class B Warrants
In connection with its issuance of common stock in public offerings that closed on November 29, 2019, the Company issued 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 stock.

The Class B warrants were immediately exercisable upon issuance, had an initial exercise price of $15.00 per share, which in accordance with the Class B warrant agreement was subsequently reduced to the price at which the Company sold its common shares in public offering or private placements. The Class B warrants expired in accordance with the Class B warrant agreement on December 29, 2022, which was 30 calendar days from the date on which the Company issued a press release announcing top-line data from its Phase 3 clinical trial of mavorixafor for the treatment of patients with WHIM syndrome.

Q2 2022 PIPE Warrants
In connection with its issuance of common stock and prefunded warrants in a private placement that closed on July 6, 2022, the Company issued warrants to purchase an aggregate of 50,925,365 warrants (the “Warrants”) for the purchase of shares of common stock. Each Warrant has an exercise price equal to $1.095 per share and will expire on the date that is 60 months from their original issue date.

Class C Warrants
In connection with its issuance of common stock in public offerings that closed on December 9, 2022, the Company issued 65,525,894 Class C warrants, which are exercisable at two Class C warrants for one share of the Company's common stock or prefunded warrants to purchase shares of the Company's common stock. The Class C warrants have an exercise price of $1.50 per set of two Class C warrants, expire on December 9, 2027 and were immediately exercisable upon issuance.

Pre-funded Warrants
In connection with the sale of its common stock in public offerings and private placements, the Company has issued pre-funded warrants to purchase shares of its common stock. The price per pre-funded warrant represents the price per share sold in the public offering or private placement, minus a nominal exercise price of either $0.001 or $0.01 per share, in accordance with the applicable pre-funded warrant agreement. The pre-funded warrants are exercisable, subject to certain beneficial ownership restrictions, at any time after their original issuance and will not expire.

The following table provides a roll forward of outstanding warrants and pre-funded warrants for the purchase of shares of the Company's common stock for the three years ended December 31, 2023:

	Number of warrants	Weighted Average Exercise Price	Weighted Average Contractual Term (Years)
Outstanding and exercisable as of December 31, 2020	13,354,403	$ 13.52	3.72
Issued	2,058,032		
Exercised	(2,130,000)		
Expired	(25,275)		
Outstanding and exercisable as of December 31, 2021	13,257,160	$ 7.96	2.72
Issued	104,531,257		
Exercised	(500,100)		
Expired	(5,416,567)		
Outstanding and exercisable as of December 31, 2022	111,871,750	$ 1.86	4.53
Issued	8,263,157		
Exercised	(7,475,814)		
Outstanding and exercisable as of December 31, 2023	112,659,093	$ 1.88	3.53

As of December 31, 2023, the Company's outstanding warrants and pre-funded warrants to purchase shares of common stock consisted of the following:

Issuance Date	Number of Shares of Common Stock Issuable	Exercise Price	Expiration Date
October 25, 2016	5,155	$ 19.78	October 24, 2026
December 28, 2017	115,916	$ 19.78	December 28, 2027
September 12, 2018	20,220	$ 19.78	September 12, 2028
October 19, 2018	20,016	$ 19.78	October 19, 2028
March 13, 2019	5,000	$ 19.78	March 12, 2029
April 16, 2019	3,866,154	$ 13.20	April 15, 2024
November 29, 2019	1,250,000	$ 12.00 (a)	n/a
March 23, 2021	50,000	$ 8.70 (b)	n/a
November 9, 2021	2,008,032	$ 4.98 (c)	n/a
March 3, 2022	766,666	$ 1.80 (d)	n/a
July 6, 2022	13,276,279	$ 1.095 (e)	n/a
July 6, 2022	44,075,050	$ 1.095	July 6, 2027
December 9, 2022	32,137,448	$ 1.50	December 9, 2027
December 9, 2022	6,800,000	$ 1.10 (f)	n/a
May 18, 2023	8,263,157	$ 1.52 (g)	n/a
	112,659,093		

(a) In November 2019, the Company received $11.999 per pre-funded warrant, or $21.0 million in aggregate proceeds. Each pre-funded warrant may be exercised for an additional $0.001 per pre-funded warrant; (b) In March 2021, the Company received $8.69 per pre-funded warrant, or $435 thousand in aggregate proceeds. Each pre-funded warrant may be exercised for an additional $0.01 per pre-funded warrant; (c) In November 2021, the Company received $4.97 per pre-funded warrant, or $10.0 million in aggregate proceeds. Each pre-funded warrant may be exercised for an additional $0.01 per pre-funded warrant; (d) In March 2022, the Company received $1.79 per pre-funded warrant, or $1.40 million in aggregate proceeds. Each pre-funded warrant may be exercised for an additional $0.01 per pre-funded warrant; (e) In July 2022, the Company received $1.094 per pre-funded warrant, or $14.5 million in aggregate proceeds. Each pre-funded warrant may be exercised for an additional $0.001 per pre-funded warrant; and (f) In December 2022, the Company received $1.099 per pre-funded warrant, or $7.5 million in aggregate proceeds. Each pre-funded warrant may be exercised for an additional $0.001 per pre-funded warrant; and (g) In May 2023, the Company received $1.519 per prefunded warrant, or $12.6 million in aggregate proceeds. Each pre-funded warrant may be exercised for an additional $0.001 per pre-funded warrant.

12. STOCK-BASED COMPENSATION

Summary of Plans— The Company issues stock awards under the following plans: (a) The 2015 Employee, Director and Consultant Equity Incentive Plan, as amended (the "2015 Plan"), (b) the Amended and Restated 2017 Equity Incentive Plan (the "2017 Plan"), (c) the Amended and Restated 2017 Employee Stock Purchase Plan (the "2017 ESPP") and the 2019 Inducement Equity Incentive Plan (the "2019 Plan").

These plans are administered by the Board of Directors or by a committee thereof. 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—Under the 2015 Plan, the Company grants incentive stock options, nonqualified stock options, restricted stock awards and other stock-based awards to employees, directors and consultants of the Company. As of December 31, 2023, there were approximately 30 thousand shares available for issuance under the 2015 Plan.

2017 Equity Incentive Plan— Under the 2017 Plan, the Company may grant incentive stock options, non-qualified options, stock appreciation rights, restricted stock awards, restricted stock units and other stock-based awards. Under an "evergreen" provision of the 2017 Plan, shares of common stock reserved for issuance under the 2017 Plan are increased annually on the first day of each year, beginning on January 1, 2021 and ending on January 1, 2027, in an amount equal to the lower of 4.0% of the number of shares of the Company's common stock outstanding on January 1 of each year or an amount determined by the Company's Board of Directors. As of December 31, 2023, approximately 181 thousand shares were available for future issuance under the 2017 Plan. As of January 1, 2024, an additional 6.7 million shares became available for future issuance under the 2017 Plan under the evergreen provision.

Amended and Restated 2017 Employee Stock Purchase Plan— The 2017 ESPP 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, 2023, 257,963 shares of common stock were issued under the 2017 ESPP. As of December 31, 2023, approximately 4.9 million shares were available for future issuance under the 2017 ESPP.

2019 Inducement Equity Incentive Plan— On June 17, 2019, the Board of Directors approved the adoption of the 2019 Plan, as amended, which is used exclusively for the grant of equity awards to individuals who were not previously employees of the Company (or following a bona fide period of non-employment), as an inducement material to such individual's entering into employment with the Company, pursuant to Nasdaq Listing Rule 5635(c)(4). The total number of shares of common stock that may be issued under the 2019 Plan, as amended, is 6.3 million 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, 2023, approximately 1.5 million 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:

	Year Ended December 31,		
	2023	2022	2021
Risk-free interest rate	4.1 %	3.4 %	1.0 %
Expected term (in years)	6.0	6.1	6.0
Expected volatility	93.9 %	96.0 %	97.3 %
Expected dividend yield	— %	— %	— %

Stock Options

The following table summarizes the Company's stock option activity for the twelve months ended December 31, 2023:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Contractual Term (in Years)	Aggregate Intrinsic Value (in Thousands)
Outstanding as of December 31, 2022	2,021,480	$ 6.99	6.5	$ 1
Granted	4,217,650	1.24		
Exercised	(2,031)	1.50		
Forfeited	(228,558)	6.60		
Outstanding as of December 31, 2023	6,008,541	$ 2.97	8.6	$ 24
Exercisable as of December 31, 2023	1,313,306	$ 8.62	5.8	$ —
Vested and expected to vest as of December 31, 2023	4,563,685	$ 3.49	8.4	$ 16

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 to the extent the stock option had a lower exercise price. The aggregate intrinsic value of stock options exercised during the twelve months ended December 31, 2023 and 2021 was $1 thousand and $13 thousand, respectively. There were no options exercised in 2022. The weighted average grant-date fair value per share of stock options granted during the years ended December 31, 2023, 2022 and 2021 was $0.97, $1.38, and $5.17, respectively.

Restricted Stock Units— During the year ended December 31, 2023, the Company granted 5.3 million restricted stock units to employees and members of the Board of Directors at a weighted average grant date fair value of $1.76 per share. Approximately 4.9 million of these awards are performance restricted stock units ("PRSUs") and the remainder are time-based and vest as the employee provides services to the Company. The PRSUs vest 50% based on the Company's achievement of each of two operational milestones conditioned on the grantee's continued employment with the Company. As of December 31, 2023, one of the two performance criteria was met. The Company believes that the achievement of the operational milestones is probable and, accordingly, stock-based compensation expense has been recognized for these awards using the accelerated attribution model, net of estimated forfeitures, based on the fair value of the awards as of the date of grant and management's best estimate of the date each operational milestone will be achieved. The Company updates its estimates related to the probability and timing of achievement of the operational milestones each period until the award either vests or is forfeited. The Company recognizes stock-based compensation expense for time-based restricted stock units ratably, net of estimated forfeitures, over the estimated vesting period.

The following table summarizes the Company's restricted stock activity for the twelve months ended December 31, 2023 :

	Number of Shares
Unvested at December 31, 2022	1,680,563
Granted	5,332,051
Vested	(3,511,542)
Forfeited	(382,248)
Unvested at December 31, 2023	3,118,824

Stock-Based Compensation— As of December 31, 2023, total unrecognized compensation expense related to unvested stock options and restricted stock units was $4.3 million, which is expected to be recognized over a weighted average period of 2.5 years.

Stock-based compensation expense was classified in the consolidated statements of operations as follows:

	Year Ended December 31,		
(in thousands)	2023	2022	2021
Research and development expense	$ 4,357	$ 2,534	$ 2,723
Selling, general and administrative expense	4,330	2,665	3,457
Total stock-based compensation	$ 8,687	$ 5,199	$ 6,180

Stock Appreciation Rights— On November 7, 2022 (the "Grant Date"), the compensation committee of the Board of Directors approved special retention and recognition grants of stock appreciation rights pursuant to the 2017 Plan to the Company's President and Chief Executive Officer, the Company's Chief Financial Officer and Treasurer, and certain other executive officers of the Company. The SARs have a measurement price per SAR equal to $1.80, the closing price per share of the Company's common stock on the Grant Date, and each grant of SARs has a maximum term of ten years from the Grant Date. Unless otherwise determined by the Board of Directors, the SARs will be settled in cash upon exercise. The settlement value will be based on the difference between the closing price of the Company's common stock on the date of settlement less $1.80 multiplied by the number of SARs exercised. The SARs will vest and become exercisable in equal annual installments on the first, second, and third anniversaries of the Grant Date, subject to the recipient remaining an employee of the Company through and including each applicable vesting date.

The calculation of the fair value of the outstanding SARs includes the closing price of the Company's common stock of $0.84 and $0.99 as of December 31, 2023 and December 31, 2022, respectively, and the following assumptions on a weighted average basis:

	December 31, 2023	December 31, 2022
Risk-free interest rate	3.8 %	4.0 %
Expected term (in years)	4.86	5.86
Expected volatility	100.0 %	97.0 %
Expected dividend yield	— %	— %
Expected forfeiture rate	18.6 %	22.3 %

The SARs are accounted for as liability awards as settlement will be in the form of cash unless the Board of Directors authorizes settlement in shares of the Company's common stock and such shares are available to be issued from the 2017 Plan. The Company currently intends to settle the SARs in cash if and when exercised. Compensation expense is recorded based the fair value of the SARs, as determined using the Black-Scholes option valuation model, using an accelerated attribution method as the SARs vest. The Company remeasures the fair value of the outstanding SARs each period until settlement and adjusts life-to-date compensation expense to the period end SARs fair value. For the years ended December 31, 2023 and 2022, the Company recognized $1.9 million and $0.4 million, respectively, of compensation expense related to the SARs.

13. INCOME TAXES

During the years ended December 31, 2023, 2022, and 2021, 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 overall tax provision for each of the three years ended December 31, 2023 primarily related to its Austrian subsidiary and Security Corp subsidiary.

Loss before the provision for income taxes for the years ended December 31, 2023, 2022 and 2021 consisted of the following:

	Year Ended December 31,		
(in thousands)	2023	2022	2021
United States	$ (102,126)	$ (94,742)	$ (89,865)
Foreign (Austria)	1,037	903	1,186
	$ (101,089)	$ (93,839)	$ (88,679)

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,		
	2023	2022	2021
U.S. federal statutory income tax rate	(21.0)%	(21.0)%	(21.0)%
State income taxes, net of federal benefit	(6.0)	(5.5)	(5.4)
Research and development tax credits	(1.4)	(1.0)	(0.9)
Other permanent differences	(0.2)	3.0	3.7
Change in deferred tax asset valuation allowance	29.3	23.2	23.7
Other	(0.7)	1.3	(0.1)
Effective income tax rate	— %	— %	— %

Net deferred tax assets as of December 31, 2023 and 2022 consisted of the following:

	December 31,	
(in thousands)	2023	2022
Net operating loss carryforwards	$ 122,942	$ 107,296
Tax credit carryforwards	8,416	6,694
Capitalized research and development expenses	28,402	16,004
Lease liabilities	755	1,016
Other	4,616	3,604
Total deferred tax assets	165,131	134,614
Valuation allowance	(163,994)	(133,112)
Deferred tax assets, net of valuation allowance	$ 1,137	$ 1,502
Right of use assets	1,137	1,502
Total deferred tax liabilities	$ 1,137	$ 1,502
Total deferred tax assets, net	$ —	$ —

As of December 31, 2023, the Company had U.S. federal and state net operating loss carryforwards of $400.0 million and $389.0 million, respectively, which may be available to offset future taxable income and begin to expire in 2031 and 2035, respectively. The Company has federal net operating losses $345.9 million, which do not expire, and $54.1 million of federal net operating losses generated prior to 2018 that will expire at various dates through 2037. In addition, as of December 31, 2023, the Company had foreign net operating loss carryforward of $62.7 million, which do not expire but are generally limited in their usage to an annual deduction equal to 75% of taxable income. As of December 31, 2023, the Company also had U.S. federal and state research and development tax credit carryforwards of $6.5 million and $2.5 million, respectively, which may be available to offset future tax liabilities and each begin to expire in 2032 and 2030, respectively.

The Tax Cuts and Jobs Act (the “Act”) was enacted on December 22, 2017. Under the Act, research and experimental expenditures incurred for tax years beginning after December 31, 2021 must be capitalized and amortized ratably over five or

fifteen years for tax purposes, depending on where the research activities are conducted. If the requirement to capitalize Section 174 expenditures is not modified, it may also impact our effective tax rate and our cash tax liability in future years.

As of December 31, 2023, uncertain tax position reserves recorded were $0.2 million for U.S. federal and state research and development tax credits.

The following table summarizes the Company's reserve for uncertain tax positions for the three years ended December 31, 2023:

(in millions)	Reserve for Uncertain Tax Position
Balance as of December 31, 2020	$ 0.3
Settlement of unrecognized tax benefit	(0.1)
Balance as of December 31, 2021	$ 0.2
Balance as of December 31, 2022	$ 0.2
Balance as of December 31, 2023	$ 0.2

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 its federal, state and foreign 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, 2023, 2022 and 2021.

Changes in the valuation allowance for deferred tax assets during the years ended December 31, 2023, 2022 and 2021 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)	2023	2022	2021
Valuation allowance, beginning of year	$ (133,112)	$ (111,835)	$ (92,197)
Increases recorded to income tax provision	(30,882)	(21,277)	(19,638)
Valuation allowance, end of year	$ (163,994)	$ (133,112)	$ (111,835)

The Company's U.S. federal and state income tax returns are generally subject to tax examinations for the tax years ended December 31, 2020 through December 31, 2022. 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.

14. NET LOSS PER SHARE

Basic and diluted net loss per share attributable to common stockholders was calculated as follow:

	Year Ended December 31,		
(in thousands, except share and per share data)	**2023**	**2022**	**2021**
Numerator:			
Net loss	$ (101,167)	$ (93,867)	$ (88,696)
Deemed dividend as a result of Class B Warrant price reset	—	(2,546)	(13,943)
Net loss attributable to common stockholders	$ (101,167)	$ (96,413)	$ (102,639)
Denominator:			
Weighted average shares of common stock—basic and diluted	177,812,480	63,525,845	25,748,797
Net loss per share attributable to common stockholders— basic and diluted	$ (0.57)	$ (1.52)	$ (3.99)

Basic and diluted weighted average shares of common stock outstanding for the years ended December 31, 2023, 2022 and 2021 includes the weighted average effect of 32.4 million, 24.2 million and 3.8 million pre-funded warrants, for the purchase of shares of common stock, for which the remaining unfunded exercise price is less than or equal to $0.01 per share. During the years ended December 31, 2022 and 2021, in accordance with the Class B Warrant agreement, the exercise price of each outstanding Class B Warrant was adjusted to the price of subsequent sales of common stock. Such adjustments are accounted for as a deemed dividend that adjusts net loss available to common shareholders for purposes of basic earnings per share. The deemed dividend was calculated using the Black-Scholes pricing model, taking into account historical volatility of the Company's common stock and the estimated remaining life of the outstanding Class B Warrants.

The Company's potentially dilutive securities included outstanding stock options, unvested restricted stock units and warrants to purchase shares of common stock for the three years ended December 31, 2023. 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 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,		
	2023	**2022**	**2021**
Options to purchase common stock	6,008,541	2,021,480	1,916,051
Unvested restricted stock units	3,118,824	1,680,563	925,101
Warrants to purchase common stock (excluding prefunded warrants, which are included in basic shares outstanding)	80,244,959	87,720,773	9,449,128
	89,372,324	91,422,816	12,290,280

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Board of Directors

Paula S. Ragan, Ph.D.
President and Chief Executive Officer

Michael S. Wyzga
Chairman of the Board of Directors

William Aliski, MPA
Director

Gary J. Bridger, Ph.D.
Director

Francoise de Craecker
Director

Alison Lawton
Director, ProQR Therapeutics N.V. and Dianthus Therapeutics

David McGirr, MBA
Director, Insmed Incorporated and Rhythm Pharmaceuticals, Inc.

Murray W. Stewart, M.D.
Director

R. Keith Woods
Director, argenx, Neurogene, Rocket Pharmaceuticals and TScan Therapeutics

Executive Officers

Paula Ragan, Ph.D.
President and Chief Executive Officer

Adam S. Mostafa
Chief Financial Officer and Treasurer

Christophe Artbet-Engels, M.D., Ph.D.
Chief Medical Officer

Mark Baldry
Chief Commercial Officer

Mary DiBiase, Ph.D.
Chief Operating Officer

Art Taveras, Ph.D.
Chief Scientific Officer

Board Committees

Audit Committee
Compensation Committee
Nominating and Corporate Governance Committee

Annual Meeting
The 2024 Annual Meeting of Stockholders will be held at the Boston offices of Goodwin Procter LLP, on the day and time as set forth in the notice of the meeting, proxy statement and form of proxy that will be mailed to stockholders in advance of the meeting.

Corporate Headquarters
61 North Beacon Street
4th Floor
Boston, Massachusetts 02134

Independent Auditors
PricewaterhouseCoopers LLP
Boston, Massachusetts

Stock Exchange
X4 Pharmaceuticals, Inc.'s common shares are listed on the Nasdaq Capital Market under the trading symbol "XFOR."

Transfer Agent
Computershare Trust Company, N.A.
150 Royall Street, Suite 101
Canton, MA 02021
(855) 879-3967
https://www.computershare.com/us

Form 10-K Report
The Company's Annual Report on Form 10-K for the year ended December 31, 2023, as filed with the Securities and Exchange Commission, is printed as part of this Annual Report. Additional copies are available without charge upon written request to:

Attention: Corporate Secretary
X4 Pharmaceuticals, Inc.
61 North Beacon Street, 4th Floor Boston, MA 02134

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