X4 PHARMACEUTICALS, INC.

2025 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 annual period ended December 31, 2025

or

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

For the transition period from _____ to _____

Commission File Number: 001-38295

X4 PHARMACEUTICALS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**27-3181608**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
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, par value $0.001 per share	**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 (§ 232.405 of this chapter) during the preceding 12 months (or 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 and 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). Yes ☐ No ☒

On June 30, 2025, the aggregate market value of the registrant's voting common stock held by non-affiliates of the registrant was approximately $14.9 million based upon the closing sale price on the Nasdaq Capital Market reported on June 30, 2025. 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 12, 2026, the registrant had 90,919,696 shares of common stock, $0.001 par value per share, outstanding.

Documents Incorporated by Reference

Portions of the registrant's definitive proxy statement, (the "2026 Proxy Statement") for its 2026 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, 2025, are incorporated by reference into Part III of this Annual Report on Form 10-K.

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EXPLANATORY NOTE

On April 28, 2025, X4 Pharmaceuticals, Inc. (the "Company"), effected a 1-for-30 reverse stock split of its common stock (the "Reverse Stock Split"). Unless otherwise noted, all references to common stock share and per share amounts in this Annual Report on Form 10-K have been retroactively adjusted to reflect the Reverse Stock Split.

See Note 14, "Common Stock and Preferred Stock" of the consolidated financial statements for a description of the Reverse Stock Split.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K 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"). All statements, other than statements of historical facts, contained in this Annual Report on Form 10-K, including statements regarding our plans, objectives, goals, strategies, future events, future operations, future financial position, future revenues or performance, projected costs, prospects, expectations, plans or intentions relating to clinical development, product candidates, the regulatory approval process, products and markets, and business trends and other information referred to in the sections titled "Business," "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations," are forward-looking statements. In some cases, 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 and 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:

- our ability to raise additional capital or achieve sufficient revenue to properly fund our business and operating plan as well as our ability to continue as a going concern;
- our expectations and goals for commercialization of XOLREMDI® (mavorixafor), which has been approved for use as an oral, once-daily therapy to increase the number of circulating mature neutrophils and lymphocytes in patients 12 years of age and older with WHIM (warts, hypogammaglobulinemia, infections, and myelokathexis) syndrome in the U.S., and that XOLREMDI, our one product approved for commercial sale, upon which we depend almost entirely to produce revenue, faces an unknown market size and growth potential, and we have not generated significant revenue from product sales to date, and we may never achieve profitability;
- 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 XOLREMDI or any of our product candidates;
- the timing of and our ability to obtain and maintain regulatory approval of our existing product XOLREMDI or any product candidates that we may develop in the future, and any related restrictions, limitations, or warnings in the label of any approved product candidates;
- our plans to research, develop, manufacture and commercialize XOLREMDI or 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 XOLREMDI and our product candidates, if approved, and the rate and degree of market acceptance of XOLREMDI and our product candidates, including reimbursement that may be received from payors;
- the benefits of U.S. Food and Drug Administration ("FDA") and European Commission ("EC") designations, including, without limitation, Fast Track, Orphan Drug 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 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 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.

PART I

In this Annual Report on Form 10-K, unless context otherwise requires or where otherwise indicated, the terms "X4" "we," "us," "our," and the "Company," refer to X4 Pharmaceuticals, Inc. and its subsidiaries.

Item 1. Business

Overview

We are a biopharmaceutical company developing and commercializing novel therapeutics for the treatment of rare hematology diseases. Our goal is to build a profitable company by generating income from products we develop and commercialize, either alone or with partners.

Our primary asset is mavorixafor, a small molecule delivered in a capsule for oral dosing as a selective antagonist of the chemokine receptor, CXCR4. We have one commercially approved product, XOLREMDI® (mavorixafor), which has received accelerated approval in the United States from the FDA for use as an oral, once-daily therapy in patients 12 years of age and older with WHIM (warts, hypogammaglobulinemia, infections, and myelokathexis) syndrome, to increase the number of circulating mature neutrophils and lymphocytes. WHIM syndrome is a rare combined primary immunodeficiency and chronic neutropenic disorder. Mavorixafor is currently being studied in a pivotal clinical trial in chronic neutropenia ("CN").

We are focused on the continued progress of our global, pivotal Phase 3 clinical trial (the "4WARD" trial) to evaluate the efficacy, safety, and tolerability of oral, once-daily mavorixafor (with or without stable doses of granulocyte colony-stimulating factor ("G-CSF")) in people with congenital, acquired primary autoimmune, or idiopathic CN who are experiencing recurrent and/or serious infections. The 52-week trial is a randomized, double-blind, placebo-controlled, multicenter study aiming to enroll up to 176 patients, with full enrollment targeted in the third quarter of 2026. The FDA has granted Fast Track designation to mavorixafor for the treatment of CN.

We are committed to making XOLREMDI available to patients in need in the U.S. while prioritizing our long-term strategy to successfully complete the 4WARD trial in patients with moderate and severe CN.

Management and Strategic Changes

In August 2025, in connection with the August PIPE Transaction (as defined below), our former President and Chief Executive Officer, Paula Ragan, Ph.D., and former Chief Financial Officer, Adam Mostafa, stepped down from their respective roles. Dr. Ragan also resigned from the Company's Board of Directors (the "Board"), and Michael Wyzga transitioned from Board Chair to Lead Independent Director. The Board appointed Adam R. Craig, M.D., Ph.D, MBA as Executive Chairman, John Volpone as President and subsequently as Chief Operating Officer, and David Kirske as Chief Financial Officer.

During 2025, we implemented two strategic restructuring actions designed to sharpen operational focus and align resources with our long-term strategy to successfully complete the 4WARD trial in patients with moderate and severe CN. These strategic restructuring actions, which resulted in an approximately 65% reduction in head count, together with certain equity financing transactions, as described below under Funding Activity, have provided us with the liquidity to pursue our strategic goals.

Funding Activity

During the year ended December 31, 2025, we completed two significant capital-raising events. In the third quarter of 2025, we sold shares of our common stock and pre-funded warrants to purchase common stock in a private placement that resulted in net proceeds of $81.0 million, after deducting placement agent fees and other expenses (the "August PIPE Transaction"). In the fourth quarter of 2025, we sold shares of common stock and pre-funded warrants to purchase common stock in a public offering that resulted in aggregate net proceeds of $145.6 million. As of December 31, 2025, we had $253.0 million in cash, cash equivalents and marketable securities.

Our Strategy

Our objective is to become a leader in the development and commercialization of novel targeted therapeutics for the treatment of rare hematology diseases. The key elements of our near-term strategy to achieve these objectives include:

- **Fully Enroll our Pivotal 4WARD Trial:** Our plan to achieve full enrollment of 176 patients on our pivotal 4WARD trial includes:
    - expanding the number of currently active clinical trial sites to over 100, including approximately 20 in the U.S.;

- enhancing our global Medical Affairs activities to increase Medical Science Liaison ("MSL") field engagement and site interaction to educate physicians on our 4WARD trial and mavorixafor's potential in treating CN;
- establishing a referral pathway for physicians to assist them in finding trial sites for their CN patients;
- consolidating our Contract Research Organizations ("CROs"); and
- investing in database mining to identify potential patients.

We are targeting completion of enrollment in 4WARD in the third quarter of 2026 and expect to provide top-line data from our 4WARD trial in the second half of 2027.

- **Prepare for Potential Commercialization of Mavorixafor for the Chronic Neutropenia Indication:** We plan to independently advance mavorixafor in the CN indication in regions that we believe have clearly defined regulatory paths and commercialization strategies. We intend to also opportunistically evaluate strategic collaborations to maximize the potential commercial value of our product candidates and discovery programs. In order to successfully commercialize mavorixafor as an oral therapy for the treatment of patients with CN, we intend to utilize our medical affairs infrastructure to engage with key opinion leaders and physicians in disease awareness and education.

- **Maintain XOLREMDI Access**: Although we are not actively promoting XOLREMDI in the U.S., our strategy is to continue to provide access to XOLREMDI to patients through our specialty pharmacy in the U.S. and our global partners internationally for their commercialization efforts. XOLREMDI has received FDA approval in the U.S. for use in patients 12 years of age and older with WHIM syndrome to increase the number of circulating mature neutrophils and lymphocytes. Mavorixafor has also received a positive opinion from the European Medicines Agency's (the "EMA") Committee for Medicinal Products for Human Use ("CHMP") recommending the granting of marketing authorization, under exceptional circumstances, for the treatment of WHIM syndrome in the European Union ("EU"). The positive opinion will now be reviewed by the EC with a final approval decision anticipated in the second quarter of 2026. If approved, mavorixafor is expected to be commercialized in Europe by our partner Norgine Pharma UK Ltd. ("Norgine"), a specialist pharmaceutical company.

Product and Development Portfolio
The following table summarizes our current product and development portfolio as of the date of this report:

Mavorixafor
Mavorixafor is an orally available, small-molecule selective antagonist of chemokine receptor CXCR4. Due to its ability to increase the mobilization of mature, functional white blood cells from the bone marrow into the bloodstream, we believe that mavorixafor has the potential to benefit people with a range of immunodeficiencies – a therapeutic market that is principally served by injectable therapies frequently associated with treatment-limiting adverse events.

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 CN disorders and certain types of cancer.

Mavorixafor, approved by the FDA under the trade name XOLREMDI for the treatment of WHIM syndrome, received Breakthrough Therapy Designation, Fast Track Designation, and Rare Pediatric Designation in the U.S., and Orphan Drug Designation in both the U.S. and EU. The FDA has also granted Fast Track designation to mavorixafor for the treatment of CN.

Chronic Neutropenia Market Overview and Opportunity

CN is a primary, rare blood condition 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). In all cases, the CXCL4/CXCR12 pathway is the key regulator of neutrophil release from the bone marrow. CN disorders are characterized by increased risks of infections and cancer due to abnormally low levels of neutrophils in the body.

Patients with CN currently have few treatment options and may be treated with G-CSF, an injectable therapy approved in the U.S. We believe that there is a broad opportunity for an oral medication, such as mavorixafor, to be used as a monotherapy in CN patients who are either naive, intolerant or unresponsive to G-CSF, with the potential for G-CSF dose reduction or elimination.

Our market research with 100 hematologists and oncologists treating patients with primary CN indicates that of the approximately 57,000 patients with primary CN (identified via ICD-10 codes associated with primary CN in database of medical claims from the 1/2023 - 6/2025 time frame,) an estimated 60%, or approximately 34,000, of these patients have moderate or severe disease, which is defined as patients having absolute neutrophil counts of <1000 cells/μL. Of these moderate and severe patients, hematologists and oncologists surveyed estimated that approximately 45%, or approximately a further subset of 15,000 patients, are experiencing serious and/or recurring infections as result of their CN. We estimate that approximately 5,000 of the 15,000 patients will represent our initial target treatment population. We believe that this patient population represents a significant market opportunity based on mavorixafor's unique value proposition for the treatment of a rare disease that has very limited therapy options. Based on our independent market research, should mavorixafor be approved for the treatment of CN, we expect to initially penetrate the market with mavorixafor as both a monotherapy and as a combination therapy, where the goal is reduction of G-CSF dosing.

Clinical Trial Results to Date for Mavorixafor in Patients with Chronic Neutropenia

Phase 1b. 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 patients with CN disorders, including idiopathic, cyclic, and congenital neutropenia. Patients received a single dose of 400 mg oral mavorixafor to assess the magnitude of treatment response. In September 2022, we announced positive results from this Phase 1b clinical trial, which enrolled a total of 25 patients:

- 100% of patients responded to treatment with a single dose of 400 mg of mavorixafor, alone or dosed concurrently with G-CSF:
  - Patients achieved a mean absolute neutrophil count ("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 patients (n=14) reached normalized ANC levels (>1,500 cells per microliter):
  - When assessed as a monotherapy in patients with severe CN who were not being treated with G-CSF (n=6), a single dose of mavorixafor led to normalized ANC levels in all patients within 2 hours, with a mean ANC increase at peak of ~2,500 cells per microliter.
  - When assessed in patients with moderate or severe neutropenia, a single dose of mavorixafor in combination with G-CSF (n=8), all patients reached normalized ANC levels.
- When assessed in patients with CN with normalized ANC counts on chronic G-CSF (n=11), all patients experienced a consistent and sustained increase in ANC.
- 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.

Phase 2. An amendment to the Phase 1b clinical trial was initiated as a Phase 2 clinical trial to evaluate the use of daily oral mavorixafor with or without injectable G-CSF for up to six months in patients with CN disorders. For patients being treated with mavorixafor in combination with G-CSF, physicians had the option to reduce the dose of G-CSF starting at Month 3. The completed Phase 2 study of mavorixafor was a six-month, open-label clinical trial that enrolled a total of 23 patients diagnosed

with idiopathic, congenital, or cyclic CN.

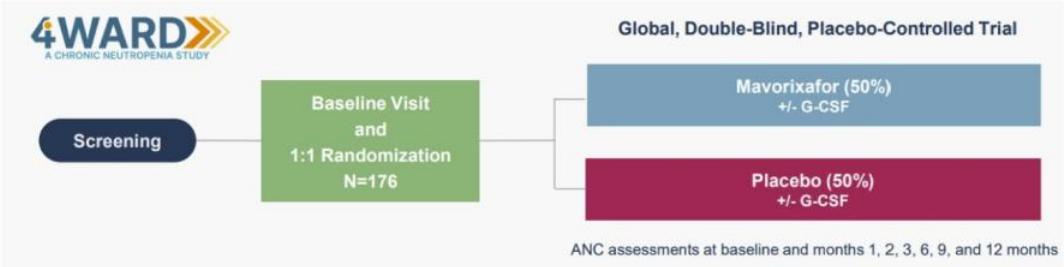
The Phase 2 study results below are from the two study treatment groups: mavorixafor monotherapy (n = 10 at baseline) and mavorixafor in combination with injectable G-CSF (n=13 at baseline).

- Mavorixafor monotherapy: Consistent with previously presented analyses, results from patients receiving mavorixafor monotherapy showed that mavorixafor durably increased mean ANC from baseline, with mean ANC reaching normal levels at Month 3 (n=9) and maintained to the end of study (Month 6 (n=8)).

- Mavorixafor in combination with injectable G-CSF: In the study, physicians chose to reduce G-CSF dosing in nine of 12 (75%) eligible patients. Of those nine, eight had G-CSF reduced at the earliest time-point permitted and three were taken completely off of G-CSF prior to their Month 6 visit.
    - Mean reductions in G-CSF were 52% at Month 3 (n=8) and 70% at Month 6 (n=9), while mean ANC levels remained in the normal range.
    - The three patients receiving mavorixafor who remained on stable doses of G-CSF maintained mean ANC levels in the normal range at all time-points.
- Safety summary: Mavorixafor was generally well tolerated as a monotherapy and in combination with G-CSF, with no drug-related serious adverse events reported, consistent with previous clinical studies.

In addition, during the Phase 2 clinical trial, a sub-study was conducted comparing the mean percentage of functional neutrophils in samples from healthy donors (n=5) to patients in the Phase 2 study (n=9) using two common study methods. These results demonstrated that the mean percentage of functional circulating neutrophils in CN patients in this sub-study was comparable to that of healthy donors after six months of mavorixafor dosing.

Phase 3 (4WARD). The 4WARD study is a Phase 3 clinical trial of mavorixafor in patients with certain CN disorders. The 4WARD trial is a global, randomized, double-blinded, placebo-controlled trial assessing the safety and efficacy of once-daily oral mavorixafor, as a monotherapy or in combination with stable doses of G-CSF, in patients with idiopathic or congenital, acquired primary autoimmune, or idiopathic CN who are experiencing recurrent and/or serious infections. The 52-week trial is expected to enroll 176 patients aged 12 years and older. Key study inclusion criteria are an ANC at baseline of less than 1,000 cells per microliter and 2 or more infections requiring intervention during the 12 months preceding the trial. The trial is designed to test for the co-primary endpoints of reduction in annualized infection rate and ANC response, defined as an increase in >500 cells per microliter in the mavorixafor-treated group versus the placebo group. Key secondary endpoints include analysis of the severity and duration of infections, antibiotic use, fatigue, and quality of life parameters. We expect to complete enrollment in the third quarter of 2026 and report top-line data results in the second half of 2027, with the goal of receiving FDA approval in 2028.



XOLREMDI® (mavorixafor) for WHIM syndrome

We have one commercially approved product, XOLREMDI® (mavorixafor), which has received accelerated approval in the United States from the FDA for use as an oral, once-daily therapy in patients 12 years of age and older with WHIM syndrome, to increase the number of circulating mature neutrophils and lymphocytes. WHIM syndrome is an ultra-rare, inherited, combined primary immunodeficiency and CN disorder.

In January 2025, we submitted a marketing authorisation application ("MAA") to the EMA seeking regulatory approval to commercialize mavorixafor for WHIM syndrome in the EU. Such MAA was validated for processing by the EMA in January 2025. In February 2026, the EMA's CHMP adopted a positive opinion recommending the grant of marketing authorization, under exceptional circumstances, for mavorixafor for the treatment of WHIM syndrome in the EU. Marketing authorization "under exceptional circumstances" is granted in situations where comprehensive clinical data cannot be obtained, including due to the rarity of the condition, and is subject to specific post-authorization obligations and annual reassessment by the EMA. The positive opinion has been submitted to the EC for review, and we expect the EC to issue a final approval decision in the second quarter of 2026.

On January 13, 2025, we announced a license and supply agreement (the "Norgine Agreement") with Norgine, pursuant to which Norgine was granted an exclusive license to distribute, market and sell our drug product for all indications, including WHIM and CN, in the European Economic Area, Switzerland, the United Kingdom, Australia, and New Zealand. In February 2025, we announced that we had entered into an agreement with Taiba Middle East Fz LLC ("taiba rare") to distribute and commercialize XOLREMDI for the treatment of WHIM syndrome in select Middle East and North African ("MENA") countries. We also have a license agreement with Abbisko Therapeutics Co. Ltd. ("Abbisko"), which granted Abbisko the exclusive right to perform regulatory activities on, manufacture and distribute mavorixafor in mainland China, Taiwan, Hong Kong and Macau.

License Agreements

License Agreement with Genzyme

In July 2014, we entered into a license agreement (the "Genzyme 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.

Regulatory Milestones: The Genzyme Agreement includes certain regulatory milestones that require us to make payments upon achievement. In January 2025, we achieved a regulatory milestone upon notification of the acceptance by the EMA of our first drug application, which triggered a payment of $3.0 million. Such payment was made in March 2025. As of December 31, 2025, we are obligated to pay Genzyme future milestone payments in the aggregate amount of up to $5.0 million, contingent upon our achievement of certain late-stage regulatory milestones with respect to licensed products, and tiered royalties based on net sales of licensed products that we commercialize under the Genzyme Agreement. The remaining regulatory milestone of $5.0 million is triggered upon the notification by the EMA of regulatory approval of our first drug application.

Sales Milestones: 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.

Sublicense Fees: We also incur fees for certain sublicense revenue that we earn from sublicensees of intellectual property ("IP") that we license from Genzyme. For example, in January 2025 we entered into the Norgine Agreement whereby we sublicensed IP, including IP that we license from Genzyme. Upon closing of the agreement, Norgine paid us a one-time, nonrefundable fee of €28.5 million for the transfer of such IP. Under the Genzyme Agreement we paid a 15% royalty to Genzyme on this upfront payment, and we may owe a 15% royalty on certain other regulatory and sales-based milestones that we earn under the Norgine Agreement.

Royalties: Upon the first sale of our drug candidate in the U.S., we incur a royalty on annual net sales at a rate of 6% up to $150 million, and would incur 10% on the portion of annual net sales between $150 million and $300 million, and 12% thereafter on annual net sales of over $300.0 million. 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 Genzyme 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 (i) the expiration of the last-to-expire valid claim of the patents licensed under the agreement that cover any licensed product, (ii) the expiration of regulatory exclusivity applicable to any licensed product and (iii) 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 a 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.

Norgine Agreement

On January 13, 2025, we entered into a license and supply agreement with Norgine. Under the terms of the Norgine Agreement, we granted Norgine the exclusive right to conduct regulatory activities on, manufacture and commercialize mavorixafor (the "Licensed Product") in Europe, Australia, and New Zealand (collectively, the "Territory"). The Norgine Agreement grants Norgine an exclusive license to import, export, promote, distribute, conduct medical affairs activities, conduct regulatory activities, have manufactured, market, advertise, offer for sale, have sold and sell the Licensed Product in the Territory and within the field. The Norgine Agreement also grants to Norgine a co-exclusive license under the Licensed IP to manufacture the Licensed Products for the Territory and within the field. We retain all rights to mavorixafor outside the Territory and specific reserved rights within the Territory. Norgine may grant sublicenses to its affiliates and certain third parties subject to the terms of the agreement, except that it may not sublicense the commercial rights granted under the agreement for certain countries without our explicit consent.

Pursuant to the terms of the Norgine Agreement, we are entitled to receive the following payments from Norgine: (i) an upfront payment in the amount of €28.5 million (such payment was received in January 2025), (ii) up to €226.0 million upon the achievement of certain regulatory, commercial and sales milestones, and (iii) escalating double-digit royalties of up to mid-twenties on any future net sales in the Territory. The tiered royalty payments are subject to royalty stacking, and to a material reduction on a country-by-country basis if a generic version of mavorixafor becomes available in the applicable country. We and Norgine will collaborate closely on regulatory filings, while we continue to be responsible for the ongoing 4WARD clinical trial evaluating mavorixafor in CN. Norgine will be responsible for all market access and commercialization activities and will eventually hold all marketing authorizations in the licensed territories. We will manufacture and supply mavorixafor to Norgine. Norgine will be required to pay a supply price for the Licensed Product derived from the contract manufacturing organization costs plus a low double-teen digit percentage of the contract manufacturing costs.

Subject to customary rights of each party to earlier terminate the Norgine Agreement, the term of the agreement continues, on a country-by-country basis, until the later of: (i) the tenth (10th) anniversary of the first commercial sale of mavorixafor, (ii) expiration of regulatory market exclusivity of mavorixafor or (iii) expiration of the last-to-expire licensed patent in such country. The term of the Norgine Agreement will be automatically renewed for additional three-year terms unless either party provides the other party with written notice of its intent not to renew the agreement at least one year prior to the applicable termination date of the agreement. In the event of automatic renewal, the royalty payment rate drops to a single digit royalty.

Patents and Other Intellectual Property Rights

We dedicate significant resources to protecting our intellectual property, which is important to our business. We have filed numerous patent applications in the United States and various other countries seeking protection of inventions originating from our research and development, and we have also obtained rights to various patents and patent applications under licenses with third parties and through acquisitions. We have pending patent applications or issued patents in the United States and foreign countries directed to mavorixafor. Patent coverage for our individual products extends for varying periods according to the date of the patent filing or grant and the legal term of patents in the various countries where we have obtained patent protection.

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.

The risks and uncertainties associated with our intellectual property, including our patents, are discussed in more detail in Part I, Item 1A, "Risk Factors."

Manufacturing, Distribution and Associated Operations

Our manufacturing strategy utilizes third-party contractors for the procurement and manufacture, as applicable, of raw materials, active pharmaceutical ingredients ("API") and finished drug product, as well as for labeling, packaging, storage and distribution of our compounds and associated supply chain operations. As a result of the April 2024 FDA approval of XOLREMDI and the continued expansion of our clinical development activities to support our 4WARD trial and, following the completion of the 4WARD trial, the potential commercial launch of mavorixafor for patients with CN, we expect that our manufacturing, distribution and related operational requirements will increase correspondingly.

Each third-party contractor undergoes a formal qualification process with our subject matter experts prior to our entry into any service agreement and initiating any manufacturing work. We currently have a master services agreement, as amended from time to time, and a commercial supply agreement with Evotec A.G. ("Evotec," previously known as Aptuit, Oxford), pursuant to which Evotec manufactures the API mavorixafor for use in our clinical and 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 December 31, 2028 and may be terminated by (i) us upon 30 days-notice to Catalent or (ii) by either party following a material breach by the other party that remains uncured for 30 days.

Integral to our manufacturing strategy is our quality control and quality assurance program, which includes standard operating procedures and specifications with the goal that our compounds are manufactured in accordance with current Good Manufacturing Practices ("cGMPs") and other applicable global regulations. The cGMP compliance includes strict adherence to regulations for quality control, quality assurance and the maintenance of records and documentation. Manufacturing facilities for products and product candidates must meet cGMP requirements, and commercialized products must have acquired FDA, EMA and any other applicable regulatory approval. In this regard, we expect to continue to rely on contract manufacturers to produce sufficient quantities of our compounds in accordance with cGMPs for use in clinical trials and distribution.

We believe our operational strategy of utilizing qualified outside vendors in the foregoing manner allows us to direct our financial and managerial resources to development and commercialization activities, rather than to the establishment and maintenance of a manufacturing and distribution infrastructure.

Competition

Competition in the pharmaceutical and biotechnology industries is intense. We face competition from a variety of companies focused on developing oncology drugs. We compete with large pharmaceutical companies and with other specialized biotechnology companies. In addition to the specific competitive factors discussed below, new anti-cancer drugs that may be developed and marketed in the future could compete with our various compounds.

Some of our existing or potential competitors have substantially greater financial, technical and human resources than us and may be better equipped to develop, manufacture and market products. Smaller companies may also prove to be significant competitors, particularly through collaborative arrangements with large pharmaceutical and established biotechnology companies. Many of these competitors have products that have been approved or are in development and operate large, well-funded research and development programs.

Companies that complete clinical trials, obtain required regulatory approvals and commence commercial sales of their products before us may achieve a significant competitive advantage if their products work through a similar mechanism as our products and if the approved indications are similar. A number of biotechnology and pharmaceutical companies are developing new products for the treatment of the same diseases being targeted by us. In some instances, such products have already entered late-stage clinical trials or received FDA or EC approval. However, cancer drugs with distinctly different mechanisms of action are often used together in combination for treating cancer, allowing several different products to target the same cancer indication or disease type. Such combination therapy is typically supported by clinical trials that demonstrate the advantage of combination therapy over that of a single-agent treatment.

We believe that our ability to compete successfully will be based on our ability to create and maintain scientifically advanced technology, develop proprietary products, attract and retain scientific personnel, obtain patent or other protection for our products, obtain required regulatory approvals and manufacture and successfully market our products, either alone or through

outside parties. We will continue to seek licenses with respect to technology related to our field of interest and may face competition with respect to such efforts. See the risk factor titled "We face direct and intense competition from our competitors in the biotechnology and pharmaceutical industries, and we may not compete successfully against them." in Part I, Item 1A, "Risk Factors" of this Annual Report on Form 10-K for additional information regarding the risks and uncertainties we face due to competition in our industry.

Government Regulation

The FDA and other regulatory authorities at federal, state and local levels, as well as in foreign countries, extensively regulate, among other things, the research, development, testing, manufacture, quality control, import, export, safety, effectiveness, labeling, packaging, storage, distribution, record keeping, approval, advertising, promotion, marketing, post-approval monitoring and post-approval reporting of drug products such as those we are developing. We, along with third-party contractors, will be required to navigate the various preclinical, clinical and commercial approval requirements of the governing regulatory agencies of the countries in which we wish to conduct studies or seek approval or licensure of our product candidates.

Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or post-marketing may subject an applicant to administrative or judicial sanctions. These sanctions could include, among other actions, the FDA's refusal to approve pending applications from the sponsor, withdrawal of an approval, a clinical hold, untitled or warning letters, product recalls or market withdrawals, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement and civil or criminal penalties. Any agency or judicial enforcement action could have a material adverse effect on our company and our products or product candidates.

U.S. Regulation

In the United States, drugs are subject to regulation under the Federal Food, Drug, and Cosmetic Act ("FDCA") and other federal, state, local, and foreign statutes and regulations. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, and local statutes and regulations requires the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or following approval may subject an applicant to administrative action and judicial sanctions. The process required by the FDA before drug product candidates may be marketed in the United States generally involves the following:

- completion of preclinical laboratory tests and animal studies performed in accordance with the FDA's current Good Laboratory Practices ("GLP") regulation;
- submission to the FDA of an Investigational New Drug Application ("IND"), which must become effective before clinical trials may begin and must be updated annually or when significant changes are made;
- approval by an independent institutional review board ("IRB"), or ethics committee at each clinical site before the trial is commenced;
- manufacture of the proposed drug candidate in accordance with cGMPs;
- performance of adequate and well-controlled human clinical trials in accordance with Good Clinical Practice ("GCP") requirements to establish the safety and efficacy of the proposed drug product candidate for its intended purpose;
- preparation of and submission to the FDA of an NDA, after completion of all pivotal clinical trials;
- satisfactory completion of an FDA Advisory Committee review, if applicable;
- a determination by the FDA within 60 days of its receipt of an NDA to file the application for review;
- satisfactory completion of an FDA pre-approval inspection of the manufacturing facility or facilities at which the proposed product is produced to assess compliance with cGMPs, and to assure that the facilities, methods and controls are adequate to preserve the drug product's continued safety and efficacy, and of selected clinical investigation sites to assess compliance with GCPs; and
- FDA review and approval of an NDA to permit commercial marketing of the product for particular indications for use in the United States.

Preclinical and Clinical Development

Prior to beginning any clinical trial with a product candidate in the United States, we must submit an IND to the FDA. An IND is a request for authorization from the FDA to administer an investigational new drug product to humans. The central focus of

an IND submission is on the general investigational plan and the protocol or protocols for preclinical studies and clinical trials. The IND also includes results of animal and in vitro studies assessing the toxicology, pharmacokinetics, pharmacology and pharmacodynamic characteristics of the product, chemistry, manufacturing and controls information, and any available human data or literature to support the use of the investigational product. In April 2025, the FDA published a roadmap to reduce animal testing in preclinical safety studies, including those required in INDs, with scientifically validated new approach methodologies ("NAMs"). An IND must become effective before human clinical trials may begin. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day period, raises safety concerns or questions about the proposed clinical trial. In such a case, the IND may be placed on clinical hold and the IND sponsor and the FDA must resolve any outstanding concerns or questions before the clinical trial can begin. Submission of an IND therefore may or may not result in FDA authorization to begin a clinical trial.

Clinical trials involve the administration of the investigational product to human subjects under the supervision of qualified investigators in accordance with GCPs, which include the requirement that all research subjects provide their informed consent for their participation in any clinical study. Clinical trials are conducted under protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A separate submission to the existing IND must be made for each successive clinical trial conducted during product development and for any subsequent protocol amendments. Furthermore, an independent IRB for each site proposing to conduct the clinical trial must review and approve the plan for any clinical trial and its informed consent form before the clinical trial begins at that site, and must monitor the study until completed. Regulatory authorities, the IRB or the sponsor may suspend a clinical trial at any time on various grounds, including a finding that the subjects are being exposed to an unacceptable health risk or that the trial is unlikely to meet its stated objectives. Some studies also include oversight by an independent group of qualified experts organized by the clinical study sponsor, known as a data safety monitoring board, which provides authorization for whether or not a study may move forward at designated check points based on access to certain data from the study and may halt the clinical trial if it determines that there is an unacceptable safety risk for subjects or other grounds, such as no demonstration of efficacy. There are also requirements governing the reporting of ongoing preclinical studies and clinical trials and clinical study results to public registries.

For purposes of NDA approval, human clinical trials are typically conducted in three sequential phases that may overlap.

- Phase 1. The investigational product is initially introduced into healthy human subjects or patients with the target disease or condition. These studies are designed to test the safety, dosage tolerance, absorption, metabolism and distribution of the investigational product in humans, the side effects associated with increasing doses, and, if possible, to gain early evidence on effectiveness.
- Phase 2. The investigational product is administered to a limited patient population with a specified disease or condition to evaluate the preliminary efficacy, optimal dosages and dosing schedule and to identify possible adverse side effects and safety risks. Multiple Phase 2 clinical trials may be conducted to obtain information prior to beginning larger and more expensive Phase 3 clinical trials.
- Phase 3. The investigational product is administered to an expanded patient population to further evaluate dosage, to provide statistically significant evidence of clinical efficacy and to further test for safety, generally at multiple geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall risk/benefit ratio of the investigational product and to provide an adequate basis for product approval.

In some cases, the FDA may require, or companies may voluntarily pursue, additional clinical trials after a product is approved to gain more information about the product. These so-called Phase 4 studies may be made a condition to approval of the NDA. Concurrent with clinical trials, companies may complete additional animal studies and develop additional information about the pharmacological characteristics of the product candidate, and must finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, must develop methods for testing the identity, strength, quality and purity of the final product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

A sponsor may choose, but is not required, to conduct a foreign clinical study under an IND. When a foreign clinical study is conducted under an IND, all IND requirements must be met unless waived. When the foreign clinical study is not conducted under an IND, the sponsor must ensure that the study complies with certain FDA regulatory requirements in order to use the study as support for an IND or application for marketing approval or licensure, including that the study was conducted in

accordance with GCP, including review and approval by an independent ethics committee and use of proper procedures for obtaining informed consent from subjects, and the FDA is able to validate the data from the study through an onsite inspection if the FDA deems such inspection necessary. The GCP requirements encompass both ethical and data integrity standards for clinical studies.

NDA Submission and Review

Assuming successful completion of all required testing in accordance with all applicable regulatory requirements, the results of product development, nonclinical studies and clinical trials are submitted to the FDA as part of an NDA requesting approval to market the product for one or more indications. The NDA must include all relevant data available from pertinent preclinical studies and clinical trials, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product's chemistry, manufacturing, controls, and proposed labeling, among other things. Data can come from company-sponsored clinical studies intended to test the safety and effectiveness of the product, or from a number of alternative sources, including studies initiated and sponsored by investigators. The submission of an NDA requires payment of a substantial application user fee to the FDA, unless a waiver or exemption applies.

In addition, under the Pediatric Research Equity Act ("PREA"), an NDA or supplement to an NDA must contain data to assess the safety and effectiveness of the biological product candidate for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The Food and Drug Administration Safety and Innovation Act requires that a sponsor who is planning to submit a marketing application for a drug product that includes a new active ingredient, new indication, new dosage form, new dosing regimen or new route of administration submit an initial pediatric study plan ("PSP") within sixty days after an end-of-Phase 2 meeting or as may be agreed between the sponsor and FDA. Unless otherwise required by regulation, PREA does not apply to any drug product for an indication for which orphan designation has been granted, except that the PREA will apply to an original NDA for a new active ingredient that is orphan-designated if the drug is a molecularly targeted cancer product intended for the treatment of an adult cancer and is directed at a molecular target that the FDA determines to be substantially relevant to the growth or progression of a pediatric cancer.

Within 60 days following submission of the application, the FDA reviews an NDA submitted to determine if it is substantially complete before the agency accepts it for filing. The FDA may refuse to file any NDA that it deems incomplete or not properly reviewable at the time of submission and may request additional information. In this event, the NDA must be resubmitted with the additional information. Once an NDA has been accepted for filing, the FDA's goal is to review standard applications within ten months after the filing date, or, if the application qualifies for priority review, six months after the FDA accepts the application for filing. In both standard and priority reviews, the review process may also be extended by FDA requests for additional information or clarification. The FDA reviews an NDA to determine, among other things, whether a product is safe, pure and potent and the facility in which it is manufactured, processed, packed or held meets standards designed to assure the product's continued safety, purity and potency. The FDA may convene an advisory committee to provide clinical insight on application review questions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. Before approving an NDA, the FDA will typically inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA will typically inspect one or more clinical sites to assure compliance with GCPs. If the FDA determines that the application, manufacturing process or manufacturing facilities are not acceptable, it will outline the deficiencies in the submission and often will request additional testing or information. Notwithstanding the submission of any requested additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

After the FDA evaluates an NDA and conducts inspections of manufacturing facilities where the investigational product and/or its drug substance will be produced, the FDA may issue an approval letter or a Complete Response letter. An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications. A Complete Response letter will describe all of the deficiencies that the FDA has identified in the NDA, except that where the FDA determines that the data supporting the application are inadequate to support approval, the FDA may issue the Complete Response letter without first conducting required inspections, testing submitted product lots and/or reviewing proposed labeling. In issuing the Complete Response letter, the FDA may recommend actions that the applicant might take to place the NDA in condition for approval, including requests for additional information or clarification. The FDA may delay or refuse approval of an NDA if applicable regulatory criteria are not satisfied, require additional testing or information and/or require post-marketing testing and surveillance to monitor safety or efficacy of a product.

If regulatory approval of a product is granted, such approval will be granted for particular indications and may entail limitations on the indicated uses for which such product may be marketed. For example, the FDA may approve the NDA with a REMS to ensure the benefits of the product outweigh its risks. A REMS is a safety strategy to manage a known or potential serious risk associated with a product and to enable patients to have continued access to such medicines by managing their safe use, and 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. The FDA also may condition approval on, among other things, changes to proposed labeling or the development of adequate controls and specifications. Once approved, the FDA may withdraw the product approval if compliance with pre-and post-marketing requirements is not maintained or if problems occur after the product reaches the marketplace. The FDA may require one or more Phase 4 post-market studies and surveillance to further assess and monitor the product's safety and effectiveness after commercialization, and may limit further marketing of the product based on the results of these post-marketing studies.

Expedited Development and Review Programs

The FDA offers a number of expedited development and review programs for qualifying product candidates. The fast track program is intended to expedite or facilitate the process for reviewing new products that meet certain criteria. Specifically, new products are eligible for fast track designation if they are intended to treat a serious or life-threatening disease or condition and data demonstrate the potential to address unmet medical needs for the disease or condition. Fast track designation applies to the combination of the product and the specific indication for which it is being studied. The sponsor of a fast track product has opportunities for more frequent interactions with the review team during product development and, once an NDA is submitted, the product may be eligible for priority review. A fast track product may also be eligible for rolling review, where the FDA may consider for review sections of the NDA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the NDA, the FDA agrees to accept sections of the NDA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the NDA.

Additionally, products studied for their safety and effectiveness in treating serious or life-threatening diseases or conditions may receive accelerated approval upon a determination that the product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. As a condition of accelerated approval, the FDA will generally require the sponsor to perform adequate and well-controlled post-marketing clinical studies to verify and describe the anticipated effect on irreversible morbidity or mortality or other clinical benefit. Under the Food and Drug Omnibus Reform Act of 2022, the FDA may require, as appropriate, that such studies be underway prior to approval or within a specific time period after the date of approval for a product granted accelerated approval. Products receiving accelerated approval may be subject to expedited withdrawal procedures if the sponsor fails to conduct the required post-marketing studies or if such studies fail to verify the predicted clinical benefit. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product.

A product intended to treat a serious or life-threatening disease or condition may also be eligible for breakthrough therapy designation to expedite its development and review. A product can receive breakthrough therapy designation if preliminary clinical evidence indicates that the product, alone or in combination with one or more other drugs or biologics, may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The designation includes all of the fast track program features, as well as more intensive FDA interaction and guidance beginning as early as Phase 1 and an organizational commitment to expedite the development and review of the product, including involvement of senior managers.

Fast track designation and breakthrough therapy designation do not change the standards for approval but may expedite the development or approval process. Even if a product qualifies for one or more of these programs, the FDA may later decide that the product no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

Orphan Drug Designation and Exclusivity

Under the Orphan Drug Act of 1983, the FDA may grant orphan drug designation to a product candidate intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the United States, or 200,000 or more individuals in the United States for which there is no reasonable expectation that the cost of developing and making available in the United States a drug or biologic for this type of disease or condition will be recovered from sales in the United States for that product candidate. Orphan drug designation must be requested before submitting an NDA. After the FDA grants orphan drug designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the

FDA. The orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review or approval process.

If a product that has orphan drug designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan drug exclusive approval (or exclusivity), which means that the FDA may not approve any other applications, including a full NDA, to market the same product for the same approved use or indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity by means of greater effectiveness, greater safety or providing a major contribution to patient care or if the holder of the orphan drug exclusivity cannot assure the availability of sufficient quantities of the orphan drug to meet the needs of patients with the same use or indication for which the already-approved or licensed product was approved or licensed. Orphan drug exclusivity does not prevent the FDA from approving a different drug or biologic for the same disease or condition, or the same drug or biologic 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.

A designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan drug designation. In addition, exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition.

There is some uncertainty with respect to the FDA's interpretation of the scope of orphan drug exclusivity. Historically, exclusivity was specific to the orphan indication for which the drug was approved. As a result, the scope of exclusivity was interpreted as preventing approval of a competing product. However, in 2021, the federal court in Catalyst Pharmaceuticals, Inc. v. Becerra suggested that orphan drug exclusivity covers the full scope of the orphan-designated "disease or condition" regardless of whether a drug obtained approval for a narrower use.

Combination Therapy

Combination therapy is a treatment modality that involves the use of two or more drugs to be used in combination to treat a disease or condition. If those drugs are combined in one dosage form, such as one pill, that is known as a fixed dose combination product and it is reviewed pursuant to the FDA's Combination Rule at 21 CFR 300.50. The rule provides that two or more drugs may be combined in a single dosage form when each component contributes to the claimed effects and the dosage of each component (amount, frequency, duration) is such that the combination is safe and effective for a significant patient population requiring such concurrent therapy as defined in the labeling for the drug.

But not all combination therapy falls under the category of a fixed dose combination. For example, the FDA recognizes that two drugs in separate dosage forms and in separate packaging, that otherwise might be administered as monotherapy for an indication, also may be used in combination for the same indication. In 2013, the FDA issued guidance to assist sponsors that were developing the range of combination therapies that fall outside the category of fixed dose combinations. That guidance provides recommendations and advice on such topics as: (1) assessment at the outset whether two or more therapies are appropriate for use in combination; (2) guiding principles for nonclinical and clinical development of the combination; (3) options for regulatory pathways to seek marketing approval of the combination; and (4) post-marketing safety monitoring and reporting obligations. Given the wide range of potential combination therapy variations, the FDA indicated it intends to assess each potential combination on a case-by case basis and encouraged sponsors to engage in early and regular consultation with the relevant review division at the agency throughout the development process for its proposed combination.

Post-Approval Requirements

Any products manufactured or distributed by us pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to record-keeping, reporting of adverse experiences, periodic reporting, product sampling and distribution, and advertising and promotion of the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims, are subject to prior FDA review and approval. There also are continuing user fee requirements, under which the FDA assesses an annual program fee for each product identified in an approved NDA. Drug manufacturers and their subcontractors are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMPs, which impose certain procedural and documentation requirements upon us and our third-party manufacturers. Changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior FDA approval before being implemented.

FDA regulations also require investigation and correction of any deviations from cGMPs and impose reporting requirements upon us and any third-party manufacturers that we may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMPs and other aspects of regulatory compliance.

The FDA may withdraw approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical studies to assess new safety risks; or imposition of distribution restrictions or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of a product, complete withdrawal of the product from the market or product recalls;
- fines, warning letters or holds on post-approval clinical studies;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of existing product approvals;
- product seizure or detention, or refusal of the FDA to permit the import or export of products;
- consent decrees, corporate integrity agreements, debarment or exclusion from federal healthcare programs;
- mandated modification of promotional materials and labeling and the issuance of corrective information;
- the issuance of safety alerts, Dear Healthcare Provider letters, press releases and other communications containing warnings or other safety information about the product; or
- injunctions or the imposition of civil or criminal penalties.

The FDA closely regulates the marketing, labeling, advertising and promotion of drugs. A company can make only those claims relating to safety and efficacy that are approved by the FDA and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. Failure to comply with these requirements can result in, among other things, adverse publicity, warning letters, corrective advertising and potential civil and criminal penalties. Physicians may prescribe legally available products for uses that are not described in the product's labeling and that differ from those tested by us and approved by the FDA. Such off-label uses are common across medical specialties. Physicians may believe that such off-label uses are the best treatment for many patients in varied circumstances. The FDA does not regulate the behavior of physicians in their choice of treatments. The FDA does, however, restrict manufacturer's communications on the subject of off-label use of their products.

Abbreviated New Drug Applications for Generic Drugs

In 1984, with passage of the Drug Price Competition and Patent Term Restoration Act of 1984 (commonly referred to as the "Hatch-Waxman Amendments") amending the Federal Food, Drug, and Cosmetic Act ("FDCA"), Congress authorized the FDA to approve generic drugs that are the same as drugs previously approved by the FDA under the NDA provisions of the statute. To obtain approval of a generic drug, an applicant must submit an abbreviated new drug application ("ANDA") to the agency. Upon approval of an ANDA, the FDA indicates that the generic product is "therapeutically equivalent" to the drug product previously approved under an NDA, known as the reference listed drug ("RLD"), and it assigns a therapeutic equivalence rating to the approved generic drug in its publication "Approved Drug Products with Therapeutic Equivalence Evaluations," also referred to as the "Orange Book." Physicians and pharmacists consider the therapeutic equivalence rating to mean that a generic drug is fully substitutable for the RLD. In addition, by operation of certain state laws and numerous health insurance programs, the FDA's designation of a therapeutic equivalence rating often results in substitution of the generic drug without the knowledge or consent of either the prescribing physician or patient.

Under the Hatch-Waxman Amendments, the FDA may not approve an ANDA until any applicable period of nonpatent exclusivity for the RLD has expired. The FDCA provides a period of five years of data exclusivity for NDAs containing a new chemical entity. In cases where such exclusivity has been granted, an ANDA may not be filed with the FDA until the expiration of five years unless the submission is accompanied by a Paragraph IV certification (discussed further below), in which case the applicant may submit its application four years following the original product approval. The FDCA also provides for a period of three years of exclusivity if the NDA includes reports of one or more new clinical investigations, other than bioavailability or bioequivalence studies, that were conducted by or for the applicant and are essential to the approval of the application. This three-year exclusivity period often protects changes to a previously approved drug product, such as a new dosage form, route of administration, combination or indication.

Hatch-Waxman Patent Certification and the 30 Month Stay

Upon approval of an NDA or a supplement thereto, NDA sponsors are required to list with the FDA each patent with claims that cover the applicant's product or a method of using the product. When an ANDA applicant files its application with the FDA, the applicant is required to certify to the FDA concerning any patents listed for the reference product in the Orange Book, except for patents covering methods of use for which the ANDA applicant is not seeking approval.

A certification that the new product will not infringe the already approved product's listed patents or that such patents are invalid or unenforceable is called a Paragraph IV certification. If the applicant does not challenge the listed patents or indicate that it is not seeking approval of a patented method of use, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired. If the ANDA applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders once the ANDA has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days after the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months, expiration of the patent, settlement of the lawsuit or a decision in the infringement case that is favorable to the ANDA applicant.

505(b)(2) New Drug Applications

As an alternative path to FDA approval for modifications to formulations or uses of products previously approved by the FDA pursuant to an NDA, an applicant may submit an NDA under Section 505(b)(2) of the FDCA. Section 505(b)(2) was enacted as part of the Hatch-Waxman Amendments and permits the filing of an NDA where at least some of the information required for approval comes from studies not conducted by, or for, the applicant, and for which the applicant has not obtained a right of reference. If the 505(b)(2) applicant can establish that reliance on the FDA's previous findings of safety and effectiveness is scientifically and legally appropriate, it may eliminate the need to conduct certain preclinical studies or clinical trials of the new product. The FDA may also require companies to perform additional bridging studies or measurements, including clinical trials, to support the change from the previously approved reference drug. The FDA may then approve the new drug candidate for all, or some, of the label indications for which the reference drug has been approved, as well as for any new indication sought by the 505(b)(2) applicant.

To the extent that a Section 505(b)(2) applicant is relying on studies conducted for an already approved product, the applicant is required to certify to the FDA concerning any patents listed for the approved product in the Orange Book to the same extent that an ANDA applicant would. As a result, approval of a 505(b)(2) NDA can be stalled until all the listed patents claiming the referenced product have expired, until any non-patent exclusivity, such as exclusivity for obtaining approval of a new chemical entity, listed in the Orange Book for the referenced product has expired, and, in the case of a Paragraph IV certification and subsequent patent infringement suit, until the earlier of 30 months, settlement of the lawsuit or a decision in the infringement case that is favorable to the Section 505(b)(2) applicant.

Patent Term Extension

In the United States, after an NDA is approved, owners of relevant drug patents may apply for up to a five-year patent extension, which permits patent term restoration as compensation for the patent term lost during the FDA regulatory process. The allowable patent term extension is typically calculated as one-half the time between, the latter of the effective date of an IND and issue date of the patent for which extension is sought, and the submission date of an NDA, plus the time between NDA submission date and the NDA approval date up to a maximum of five years. The time can be shortened if the FDA determines that the applicant did not pursue licensure with due diligence. The total patent term after the extension may not exceed 14 years from the date of product approval. Only one patent applicable to an approved drug product is eligible for extension and only those claims covering the product, a method for using it, or a method for manufacturing it may be extended and the application for the extension must be submitted prior to the expiration of the patent in question. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Some, but not all, foreign jurisdictions possess patent term extension or other additional patent exclusivity mechanisms that may be more or less stringent and comprehensive than those of the U.S.

Other Healthcare Laws and Compliance Requirements

Pharmaceutical companies are subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which they conduct their business. Such laws include, without limitation: the federal Anti-Kickback Statute ("AKS"); the federal False Claims Act ("FCA"); the Health Insurance Portability and Accountability Act of 1996 ("HIPAA") and similar foreign, federal and state fraud, abuse and transparency laws.

The AKS prohibits, among other things, persons and entities from knowingly and willfully soliciting, receiving, offering or paying remuneration, to induce, or in return for, either the referral of an individual, or the purchase or recommendation of an item or service for which payment may be made under any federal healthcare program. The term remuneration has been interpreted broadly to include anything of value. The AKS has been interpreted to apply to arrangements between pharmaceutical manufacturers on one hand, and prescribers and purchasers on the other. The government often takes the position that to violate the AKS, only one purpose of the remuneration need be to induce referrals, even if there are other legitimate purposes for the remuneration. There are a number of statutory exceptions and regulatory safe harbors protecting some common commercial activities from AKS prosecution, but they are drawn narrowly and practices that involve remuneration, such as consulting agreements, for persons in a position to refer or recommend federally reimbursable healthcare business may be alleged to be intended to induce prescribing, purchasing or recommending, and may be subject to scrutiny if they do not qualify for an exception or regulatory safe harbor. Qualifying for a statutory exception or regulatory safe harbor requires satisfying all of the criteria for the exception or safe harbor. Our practices may not in all cases meet all of the criteria for protection under a statutory exception or regulatory safe harbor. Failure to meet all of the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal under the AKS, but it does increase the risk of regulatory scrutiny. Ultimately, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all of its facts and circumstances. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation.

The FCA, which can be enforced through civil whistleblower or qui tam actions, prohibits, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment of federal government funds, including in federal healthcare programs, that are false or fraudulent. Pharmaceutical and other healthcare companies have been prosecuted under these laws for engaging in a variety of different types of conduct that caused the submission of false claims to federal healthcare programs. Under the AKS, for example, a claim resulting from a violation of the AKS is deemed to be a false or fraudulent claim for purposes of the FCA.

HIPAA created additional federal criminal statutes that prohibit, among other things, executing a scheme to defraud any healthcare benefit program, including private third-party payors, and making false statements relating to healthcare matters. A person or entity does not need to have actual knowledge of the healthcare fraud statute implemented under HIPAA or specific intent to violate the statute in order to have committed a violation.

The FDCA addresses, among other things, the design, production, labeling, promotion, manufacturing, and testing of drugs, biologics and medical devices, and prohibits such acts as the introduction into interstate commerce of adulterated or misbranded drugs or devices. The PHSA also prohibits the introduction into interstate commerce of unlicensed or mislabeled biological products.

The U.S. federal Physician Payments Sunshine Act requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to annually report to the Centers for Medicaid & Medicare Services ("CMS") information related to payments or other transfers of value to various healthcare professionals including physicians, physician assistants, nurse practitioners, clinical nurse specialists, certified nurse anesthetists, certified nurse-midwives, and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members. Beginning on January 1, 2023, California Assembly Bill 1278 requires California physicians and surgeons to notify patients of the Open Payments database established under the federal Physician Payments Sunshine Act.

We are also subject to federal price reporting laws and federal consumer protection and unfair competition laws. Federal price reporting laws 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. Federal consumer protection and unfair competition laws broadly regulate marketplace activities and activities that potentially harm consumers.
We are also subject to additional similar U.S. state and foreign law equivalents of each of the above federal laws, which, in some cases, differ from each other in significant ways, and may not have the same effect, thus complicating compliance efforts. If our operations are found to be in violation of any of such laws or any other governmental regulations that apply, we may be subject to penalties, including, without limitation, civil, criminal and administrative penalties, damages, fines, exclusion from government-funded healthcare programs, such as Medicare and Medicaid or similar programs in other countries or jurisdictions, integrity oversight and reporting obligations to resolve allegations of non-compliance, disgorgement, individual imprisonment, contractual damages, reputational harm, diminished profits and the curtailment or restructuring of our operations.

Data Privacy and Security

Numerous state, federal, and foreign laws govern the collection, dissemination, use, access to, confidentiality, and security of personal information, including health-related information. In the United States, numerous federal and state laws and regulations, including state data breach notification laws, state health information privacy laws, and federal and state consumer protection laws and regulations, govern the collection, use, disclosure, and protection of health-related and other personal information and could apply to our operations or the operations of our partners.

For example, HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act ("HITECH"), and their respective implementing regulations impose data privacy, security, and breach notification obligations on certain health care providers, health plans, and health care clearinghouses, known as covered entities, as well as their business associates and their covered subcontractors that perform certain services that involve using, disclosing, creating, receiving, maintaining, or transmitting individually identifiable protected health information (PHI) for or on behalf of such covered entities. These requirements imposed by HIPAA and HITECH on covered entities and business associates include entering into agreements that require business associates protect PHI provided by the covered entity against improper use or disclosure, among other things; following certain standards for the privacy of PHI, which limit the disclosure of a patient's past, present, or future physical or mental health or condition or information about a patient's receipt of health care if the information identifies, or could reasonably be used to identify, the individual; ensuring the confidentiality, integrity, and availability of all PHI created, received, maintained, or transmitted in electronic form, to identify and protect against reasonably anticipated threats or impermissible uses or disclosures to the security and integrity of such PHI; and reporting of breaches of PHI to individuals and regulators.

Entities that are found to be in violation of HIPAA may be subject to significant civil, criminal, and administrative fines and penalties and/or additional reporting and oversight obligations if required to enter into a resolution agreement and corrective action plan with HHS to settle allegations of HIPAA non-compliance. A covered entity or business associate is also liable for civil money penalties for a violation that is based on an act or omission of any of its agents, which may include a downstream business associate, as determined according to the federal common law of agency. HITECH also increased the civil and criminal penalties applicable to covered entities and business associates and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce HIPAA and seek attorneys' fees and costs associated with pursuing federal civil actions. To the extent that we submit electronic healthcare claims and payment transactions that do not comply with the electronic data transmission standards established under HIPAA and HITECH, payments to us may be delayed or denied.

In addition, state health information privacy laws, such as California's Confidentiality of Medical Information Act and Washington's My Health My Data Act, that govern the privacy and security of health-related information, specifically, may apply even when HIPAA does not and impose additional requirements.

Even when HIPAA and state health information privacy laws do not apply, according to the FTC and state attorneys general, violating consumers' privacy rights or failing to take appropriate steps to keep consumers' personal information secure may constitute unfair acts or practices in or affecting commerce in violation of Section 5(a) of the Federal Trade Commission Act and state consumer protection laws.

In addition, certain state laws, such as the California Consumer Privacy Act of 2018 ("CCPA"), as amended by the California Privacy Rights Act of 2020, govern the privacy and security of personal information, including health-related information in certain circumstances, some of which are more stringent than HIPAA in various ways. Numerous other states have passed similar laws, but many differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts. The CCPA applies to personal data of consumers, business representatives, and employees, and imposes obligations on certain businesses that do business in California, including to provide specific disclosures in privacy notices, and affords rights to California residents in relation to their personal information. Health information falls under the CCPA's definition of personal information where it identifies, relates to, describes, or is reasonably capable of being associated with or could reasonably be linked, directly or indirectly, with a particular consumer or household and is included under a new category of personal information, "sensitive personal information," which is offered greater protection. The CCPA and numerous other comprehensive privacy laws that have passed or are being considered in other states, as well as at the federal and local levels, exempt PHI that is subject to HIPAA; and others exempt covered entities and business associates subject to HIPAA altogether, further complicating compliance efforts, and increasing legal risk and compliance costs for us and the third parties upon whom we rely.

Failure to comply with these laws, where applicable, can result in the imposition of significant civil and/or criminal penalties and private litigation. Privacy and security laws, regulations, and other obligations are constantly evolving, may conflict with each other to complicate compliance efforts, and can result in investigations, proceedings, or actions that lead to significant civil and/or criminal penalties and restrictions on data processing.

Coverage and Reimbursement

In the United States and markets in other countries, patients generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. Adequate coverage and reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and commercial payors is critical to new product acceptance. Our ability to successfully commercialize our product candidates 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. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow it to establish or maintain pricing sufficient to realize a sufficient return on its investment. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels.

Significant uncertainty exists as to the coverage and reimbursement status of any pharmaceutical or biological product for which we obtain regulatory approval. Sales of any product, if approved, depend, in part, on the extent to which such product will be covered by third-party payors, such as federal, state, and foreign government healthcare programs, commercial insurance and managed healthcare organizations, and the level of reimbursement, if any, for such product by third-party payors. Decisions regarding whether to cover any of our product candidates, if approved, the extent of coverage and amount of reimbursement to be provided are made on a plan-by-plan basis. Further, no uniform policy for coverage and reimbursement exists in the United States, and coverage and reimbursement can differ significantly from payor to payor. 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. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our product candidates to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance. Factors payors consider in determining reimbursement are based on whether the product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- cost-effective; and
- neither experimental nor investigational.

Third-party payors are increasingly challenging the prices charged for medical products and services, examining the medical necessity and reviewing the cost effectiveness of pharmaceutical or biological products, medical devices and medical services, in addition to questioning safety and efficacy. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit sales of any product that receives approval. Decreases in third-party reimbursement for any product or a decision by a third-party not to cover a product could reduce physician usage and patient demand for the product.

For products administered under the supervision of a physician, obtaining coverage and adequate reimbursement may be particularly difficult because of the higher prices often associated with such drugs. Additionally, separate reimbursement for the product itself or the treatment or procedure in which the product is used may not be available, which may impact physician utilization. In addition, companion diagnostic tests require coverage and reimbursement separate and apart from the coverage and reimbursement for their companion pharmaceutical or biological products. Similar challenges to obtaining coverage and reimbursement, applicable to pharmaceutical or biological products, will apply to companion diagnostics.

In addition, the U.S. government, state legislatures and foreign governments have continued implementing cost-containment programs, including price controls, restrictions on coverage and reimbursement and requirements for substitution of generic products. The IRA provides CMS with significant new authorities intended to curb drug costs and to encourage market competition. For the first time, CMS will be able to directly negotiate prescription drug prices and to cap out-of-pocket costs. Each year, CMS will select and negotiate a preset number of high-spend drugs and biologics that are covered under Medicare Part B and Part D that do not have generic or biosimilar competition. On August 29, 2023, HHS announced the list of the first

ten drugs subject to price negotiations. These price negotiations occurred in 2024. In January 2025, CMS announced a list of 15 additional Medicare Part D drugs that will be subject to price negotiations. The IRA also provides a new "inflation rebate" covering Medicare patients that took effect in 2023 and is intended to counter certain price increases in prescriptions drugs. The inflation rebate provision requires drug manufacturers to pay a rebate to the federal government if the price for a drug or biologic under Medicare Part B and Part D increases faster than the rate of inflation. To support biosimilar competition, beginning in October 2022, qualifying biosimilars may receive a Medicare Part B payment increase for a period of five years. Separately, if a biologic drug for which no biosimilar exists delays a biosimilar's market entry beyond two years, CMS will be authorized to subject the biologics manufacturer to price negotiations intended to ensure fair competition. Notwithstanding these provisions, the IRA's impact on commercialization and competition remains largely uncertain.

In addition, 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 U.S. 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 reimbursement will be available for any product candidate that we may commercialize and, if reimbursement is available, the level of reimbursement. In addition, many pharmaceutical manufacturers must calculate and report certain price reporting metrics to the government, such as average sales price and best price. Penalties may apply in some cases when such metrics are not submitted accurately and timely. Further, these prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs.

Finally, 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, the EU provides options for its member states to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. 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. A member state may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. 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 product candidates. Historically, products launched in the EU do not follow price structures of the U.S. and generally prices tend to be significantly lower.

Healthcare Reform

The United States and some foreign jurisdictions are considering or have enacted a number of reform proposals to change the healthcare system. There is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality or expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by federal and state initiatives, including those designed to limit the pricing, coverage, and reimbursement of pharmaceutical and biopharmaceutical products, especially under government-funded health care programs, and increased governmental control of drug pricing.

The ACA, which was enacted in March 2010, substantially changed the way healthcare is financed by both governmental and private insurers in the United States, and significantly affected the pharmaceutical industry. The ACA contains a number of provisions of particular import to the pharmaceutical and biotechnology industries, including, but not limited to, those governing enrollment in federal healthcare programs, a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, and annual fees based on pharmaceutical companies' share of sales to federal health care programs. Since its enactment, there have been judicial and Congressional challenges to certain aspects of the ACA, and we expect there will be additional challenges and amendments to the ACA in the future. For example, the 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 significantly lowering the beneficiary maximum out-of-pocket cost and creating a new manufacturer discount program.

Other legislative changes have been proposed and adopted since the ACA was enacted, including automatic aggregate reductions of Medicare payments to providers of on average 2% per fiscal year as part of the federal budget sequestration under the Budget Control Act of 2011. These reductions went into effect in April 2013 and, due to subsequent legislative amendments, will remain in effect until 2032 unless additional action is taken by Congress. In addition, the Bipartisan Budget Act of 2018, among other things, amended the Medicare Act (as amended by the ACA) to increase the point-of-sale discounts that manufacturers must agree to offer under the Medicare Part D coverage discount program from 50% to 70% off negotiated

prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs being covered under Medicare Part D.

Moreover, there has recently been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several Congressional inquiries and proposed and enacted federal and state measures designed to, among other things, reduce the cost of prescription drugs, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug products. For example, in May 2019, CMS adopted a final rule allowing Medicare Advantage Plans the option to use step therapy for Part B drugs, permitting Medicare Part D plans to apply certain utilization controls to new starts of five of the six protected class drugs, and requiring the Explanation of Benefits for Part D beneficiaries to disclose drug price increases and lower cost therapeutic alternatives, which went into effect on January 1, 2021. In May 2025, the Trump Administration renewed the idea of international reference pricing through an executive order entitled "Delivering Most-Favored-Nation Prescription Drug Pricing to American Patients," which, among other things, directs the HHS and other agencies to communicate most-favored-nation price targets to pharmaceutical manufacturers to bring prices for U.S. patients in line with comparably developed nations and to facilitate direct-to-consumer purchasing programs. The HHS subsequently issued guidance indicating the MFN target price will be the lowest price paid in an Organisation for Economic Co-operation and Development country with a gross domestic product (GDP) per capita of at least 60% of the U.S. GDP per capital. In addition, in December 2025, CMS proposed new drug payment models to lower drug prices for Medicare beneficiaries; under the models, CMS would explore potential adjustments to Medicare drug inflation rebate calculations by comparison to international drug pricing information. It is currently unclear whether and to what extent these measures will be implemented and what impact any such implementation would have on our business.

Notwithstanding the IRA, continued legislative and enforcement interest exists in the United States with respect to specialty drug pricing practices. Specifically, we expect government authorities to continue pushing for transparency to drug pricing, reducing the cost of prescription drugs under Medicare, reviewing the relationship between pricing and manufacturer patient programs, and reforming government program reimbursement methodologies for drugs.

Individual states in the U.S. have also become increasingly active in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain drug access and marketing cost disclosure and transparency measures, and designed to encourage importation from other countries and bulk purchasing. Legally mandated price controls on payment amounts by third-party payors or other restrictions could harm our business, financial condition, results of operations and prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. This could reduce the ultimate demand for its drugs or put pressure on its drug pricing, which could negatively affect our business, financial condition, results of operations and prospects.

Other Government Regulation Outside of the United States

In addition to regulations in the United States, we are subject to a variety of regulations in other jurisdictions governing, among other things, research and development, clinical trials, testing, manufacturing, safety, efficacy, quality control, labeling, packaging, storage, record keeping, distribution, reporting, export and import, advertising, marketing and other promotional practices involving biological products as well as authorization, approval as well as post-approval monitoring and reporting of our products. Because biologically sourced raw materials are subject to unique contamination risks, their use may be restricted in some countries.

Whether or not we obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Certain countries outside of the United States have a similar process that requires the submission of a clinical trial application much like the IND prior to the commencement of human clinical trials.

The requirements and process governing the conduct of clinical trials, including requirements to conduct additional clinical trials, product licensing, safety reporting, post-authorization requirements, marketing and promotion, interactions with healthcare professionals, pricing and reimbursement may vary widely from country to country. No action can be taken to market any product in a country until an appropriate approval application has been approved by the regulatory authorities in that country. The current approval process varies from country to country, and the time spent in gaining approval varies from that required for FDA approval. In certain countries, the sales price of a product must also be approved. The pricing review

period often begins after market approval is granted. Even if a product is approved by a regulatory authority, satisfactory prices may not be approved for such product, which would make launch of such products commercially unfeasible in such countries.

Regulation in the European Union

European Data Laws

The processing of personal data, including health-related personal data in the European Economic Area ("EEA") is mainly governed by the provisions of the European General Data Protection Regulation (EU) 2016/679 ("GDPR"), and related data protection laws in individual EEA countries. In the United Kingdom, the processing of personal data is mainly governed by the GDPR as incorporated into UK law pursuant to the European Union (Withdrawal) Act 2018 (the "UK GDPR"). The GDPR and UK GDPR impose a number of strict obligations and requirements for the processing, including collecting, analyzing and transferring, of personal data of individuals in the EEA or in the UK, in particular with respect to health data from clinical trials and adverse event reporting. The GDPR and UK GDPR include requirements relating to the legal basis of the processing (such as consent of the individuals to whom the personal data relates), the information provided to the individuals prior to processing their personal data, the personal data breaches which may have to be notified to the national data protection authorities and data subjects, the measures to be taken when engaging processors, and obligations relating to the security and confidentiality of the personal data. EEA countries may also impose additional requirements in relation to the processing of health, genetic and biometric data through their national legislation.

In addition, the GDPR imposes specific restrictions on the transfer of personal data to countries outside of the EEA that are not considered by the EC to provide an adequate level of data protection. Appropriate safeguards are required to enable such transfers. Among the appropriate safeguards that can be used, the data exporter may use the standard contractual clauses ("SCCs"). When relying on the appropriate safeguards, data exporters, with the assistance of the data importers, are also required to conduct a transfer risk assessment to verify if anything in the law and/or practices of the third country may impinge on the effectiveness of the safeguards in the context of the transfer at stake and, if so, to identify and adopt supplementary measures that are necessary to bring the level of protection of the data transferred to the EU standard of essential equivalence. Where no supplementary measure is suitable, the data exporter should avoid, suspend or terminate the transfer. With regard to the transfer of data from the EEA to the United States, on July 10, 2023, the EC adopted its adequacy decision for the EU-US Data Privacy Framework. On the basis of the new adequacy decision, personal data can flow from the EEA to U.S. companies participating in the framework.

With regard to the transfer of data from the EEA to the UK, based on the EC's adequacy decision of June 28, 2021 and subsequent renewals, personal data may continue to flow freely from the EEA to the UK on the basis that the UK is deemed to provide an adequate level of data protection until December 27, 2031. The adequacy decisions will automatically expire unless renewed.

With respect to transfers from the UK to other countries, these transfers are also subject to specific transfer rules under the UK regime. These UK international transfer rules broadly mirror the EU GDPR rules.

On February 2, 2022, the UK Secretary of State laid before the UK Parliament the international data transfer agreement ("IDTA") and the international data transfer addendum to the EC's standard contractual clauses for international data transfers (UK Addendum) and a document setting out transitional provisions. The IDTA and UK Addendum came into force on March 21, 2022 and are the primary UK-approved mechanisms for putting in place appropriate safeguards for UK restricted transfers, subject to transitional arrangements for legacy SCCs. Regarding transfers from the UK to the EEA, the UK Information Commissioner's Office ("ICO") guidance indicates that organizations do not need new arrangements. With regard to the transfer of personal data from the UK to the United States, the UK government has adopted an adequacy decision for the UK Extension to the EU-US Data Privacy Framework, the UK-US Data Bridge, which came into force on October 12, 2023. The UK-US Data Bridge recognizes the United States as offering an adequate level of data protection where the recipient is a U.S. organization certified to the EU-US Data Privacy Framework and participating in the UK Extension to the EU-US Data Privacy Framework.

Failure to comply with the requirements of the GDPR or UK GDPR and the related national data protection laws of the EEA countries may result in significant monetary fines for noncompliance of up to €20 million or £17.5 million (as applicable), 4% of the total worldwide annual turnover (for higher-tier infringements). This is enforced by ICO and is entirely separate from fines under EU GDPR. In addition, violations of national laws can trigger additional, administrative penalties, investigations, corrective orders, temporary or definitive bans, and, in some jurisdictions, and a number of criminal offenses for organizations

and, in certain cases, their directors and officers, as well as civil liability claims from individuals whose personal data was processed.

Data protection authorities from the different EEA countries may still implement certain variations, enforce the GDPR and national data protection laws differently, and introduce additional national regulations and guidelines, which adds to the complexity of processing personal data in the EEA.

Furthermore, there are specific requirements relating to processing health data from clinical trials, including public disclosure obligations provided in the EU Clinical Trials Regulation No. 536/2014 ("CTR"), EMA disclosure initiatives and voluntary commitments by industry. Failure to comply with these obligations could lead to government enforcement actions and significant penalties against us, harm to our reputation, and adversely impact our business and operating results.

Drug and Biologic Development Process

Regardless of where they are conducted, all clinical trials included in applications for marketing authorization ("MA") for human medicines in the EU/EEA must have been carried out in accordance with EU regulations. This means that clinical trials conducted in the EU/EEA have to comply with EU clinical trial legislation but also that clinical trials conducted outside the EU/EEA have to comply with ethical principles equivalent to those set out in the EEA, including adhering to international good clinical practice and the Declaration of Helsinki. The conduct of clinical trials in the EU is governed by the CTR, which entered into force on January 31, 2022. The CTR replaced the Clinical Trials Directive 2001/20/EC, ("Clinical Trials Directive") and introduced a complete overhaul of the existing regulation of clinical trials for medicinal products in the EU.

Under the CTR, a sponsor is able to submit a single application for approval of a clinical trial through a centralized EU clinical trials portal (the "Clinical Trials Information System" or "CTIS"). One national regulatory authority (the reporting EU member state proposed by the applicant) will take the lead in validating and evaluating the application consult and coordinate with the other concerned EU Member States. If an application is rejected, it may be amended and resubmitted through the EU clinical trials portal. If an approval is issued, the sponsor may start the clinical trial in all concerned EU Member States. However, a concerned EU member state may in limited circumstances declare an "opt-out" from an approval and prevent the clinical trial from being conducted in such member state. The CTR also aims to streamline and simplify the rules on safety reporting, and introduces enhanced transparency requirements such as mandatory submission of a summary of the clinical trial results to the EU database, including a layperson's summary. Since January 31, 2023, submission of initial clinical trial applications via CTIS is mandatory and CTIS serves as the single entry point for submission of clinical trial-related information and data. As of January 31, 2025, all ongoing trials approved under the former Clinical Trials Directive need to comply with the CTR and have to be transitioned to CTIS.

Under the CTR, national laws, regulations, and the applicable GCP and GLP standards must also be respected during the conduct of the trials, including the International Council for Harmonization of Technical Requirements for Pharmaceuticals for Human Use (ICH) guidelines on Good Clinical Practice and the ethical principles that have their origin in the Declaration of Helsinki. Under the current regime all suspected unexpected serious adverse reactions to the investigated drug that occur during the clinical trial must be reported to the National Competent Authority and to the Ethics Committees of the EU member state where they occur.

During the development of a medicinal product, the EMA and national regulators within the EU provide the opportunity for dialogue and guidance on the development program. At the EMA level, this is usually done in the form of scientific advice, which is given by the CHMP on the recommendation of the Scientific Advice Working Party ("SAWP"). A fee is incurred with each scientific advice procedure, but is significantly reduced for designated orphan medicines. Advice from the EMA is typically provided based on questions concerning, for example, quality (chemistry, manufacturing and controls testing), nonclinical testing and clinical studies, and pharmacovigilance plans and risk-management programs. Advice is not legally binding with regard to any future Marketing Authorization Application ("MAA") of the product concerned.

Drug Marketing Authorization

In the EEA, after completion of all required clinical testing, pharmaceutical products may only be placed on the market after obtaining a MA. To obtain an MA of a drug under EU regulatory systems, an applicant can submit an MAA through, amongst others, a centralized or decentralized procedure.

To be used or sold in the UK, a drug must have an effective MA granted by the Medicines and Healthcare Products Regulatory Agency ("MHRA") under the Human Medicines Regulations 2012 (SI 2012/1916), as amended. MA applications are submitted electronically via the MHRA Submissions Portal. Under the MHRA's national assessment procedure, the MHRA generally

aims to reach a decision within 210 “clock-on” days, excluding any “clock-stops” while the applicant prepares responses to MHRA questions.

On August 30, 2023, the MHRA published detailed guidance on its recently announced new International Recognition Procedure (“IRP”) for MAAs. The IRP applies since January 1, 2024 and replaces existing EU reliance procedures to apply for authorizations from seven international regulators (e.g. Health Canada, Swiss Medic, FDA, EMA, among others). The IRP allows medicinal products approved in other jurisdictions that meet certain criteria to undergo a fast-tracked MHRA review to obtain and/or update a MA in the UK. Applicants can submit initial MAAs to the IRP but the procedure can also be used throughout the lifecycle of a product for post-authorization procedures including line extensions, variations and renewals.

Centralized Authorization Procedure
The centralized procedure provides for the grant of a single MA that is issued by the EC following the scientific assessment of the application by the EMA that is valid for all EU Member States as well as in the three additional EEA Member States (Norway, Iceland and Liechtenstein). The centralized procedure is compulsory for specific medicinal products, including for medicines developed by means of certain biotechnological processes, products designated as orphan medicinal products, advanced therapy medicinal products (gene therapy, somatic cell therapy, or tissue engineered medicines) and medicinal products with a new active substance indicated for the treatment of certain diseases (HIV/AIDS, cancer, neurodegenerative disorders, diabetes, auto-immune diseases and other immune dysfunctions, and viral diseases). For medicinal products containing a new active substance not yet authorized in the EEA before May 20, 2004 and indicated for the treatment of other diseases, medicinal products that constitute significant therapeutic, scientific or technical innovations or for which the grant of a MA through the centralized procedure would be in the interest of public health at EU level, an applicant may voluntarily submit an application for a MA through the centralized procedure.

Under the centralized procedure, the CHMP is responsible for conducting the initial assessment of a drug. The CHMP is also responsible for several post-authorization and maintenance activities, such as the assessment of modifications or extensions to an existing MA. Under the centralized procedure, the timeframe for the evaluation of an MAA by the EMA’s CHMP is, in principle, 210 days from receipt of a valid MAA. However, this timeline excludes clock stops, when additional written or oral information is to be provided by the applicant in response to questions asked by the CHMP, so the overall process typically takes a year or more, unless the application is eligible for an accelerated assessment. Accelerated evaluation might be granted by the CHMP in exceptional cases, when a medicinal product is expected to be of a major public health interest, particularly from the point of view of therapeutic innovation. Upon request, the CHMP can reduce the time frame to 150 days if the applicant provides sufficient justification for an accelerated assessment. The CHMP will provide a positive opinion regarding the application only if it meets certain quality, safety and efficacy requirements. This opinion is then transmitted to the EC, which has the ultimate authority for granting MA within 67 days after receipt of the CHMP opinion.

Decentralized Authorization Procedure
Medicines that fall outside the mandatory scope of the centralized procedure have three routes to authorization: (i) they can be authorized under the centralized procedure if they concern a significant therapeutic, scientific or technical innovation, or if their authorization would be in the interest of public health; (ii) they can be authorized under a decentralized procedure where an applicant applies for simultaneous authorization in more than one EU member state; or (iii) they can be authorized in an EU member state in accordance with that state’s national procedures and then be authorized in other EU countries by a procedure whereby the countries concerned agree to recognize the validity of the original, national MA (mutual recognition procedure).

The decentralized procedure permits companies to file identical MA applications for a medicinal product to the competent authorities in various EU Member States simultaneously if such medicinal product has not received marketing approval in any EU Member State before. This procedure is available for pharmaceutical products not falling within the mandatory scope of the centralized procedure. The competent authority of a single EU Member State, the reference member state, is appointed to review the application and provide an assessment report. The competent authorities of the other EU Member States, the concerned member states, are subsequently required to grant a MA for their territories on the basis of this assessment. The only exception to this is where the competent authority of an EU Member State considers that there are concerns of potential serious risk to public health, the disputed points are subject to a dispute resolution mechanism and may eventually be referred to the EC, whose decision is binding for all EU Member States.

Risk Management Plan
All new MAAs must include a Risk Management Plan (“RMP”) describing the risk management system that the company will put in place and documenting measures to prevent or minimize the risks associated with the product. RMPs are continually modified and updated throughout the lifetime of the medicine as new information becomes available. An updated RMP must be

submitted: (i) at the request of EMA or a national competent authority, or (ii) whenever the risk-management system is modified, especially as the result of new information being received that may lead to a significant change to the benefit-risk profile or as a result of an important pharmacovigilance or risk-minimization milestone being reached. The regulatory authorities may also impose specific obligations as a condition of the MA. Since October 20, 2023, all RMPs for centrally authorized products are published by the EMA, subject only to limited redactions.

MA Validity Period

MAs have an initial duration of five years. After these five years, the authorization may subsequently be renewed on the basis of a reevaluation of the risk-benefit balance. Once renewed, the MA is valid for an unlimited period unless the EC or the national competent authority decides, on justified grounds relating to pharmacovigilance, to proceed with only one additional five-year renewal. Applications for renewal must be made to the EMA at least nine months before the five-year period expires. Any authorization which is not followed by the actual placing of the drug on the EU market (in case of centralized procedure) or on the market of the authorizing member state within three years after authorization ceases to be valid.

For the UK, the period of three years during which the drug has not been marketed in Great Britain will be restarted from the date of conversion to a Great Britain MA. Following Windsor Framework changes, which became effective January 1, 2025, EC authorizations are no longer valid in Northern Ireland and centrally authorized products are instead authorized by the MHRA under UK-wide marketing authorizations; existing licenses for product licensed by the MHRA that covers Great Britain only become geographically valid UK-wide while retaining their license number/prefix.

On the other hand, for the EU, in the case the drug has been marketed in the UK, the placing on the UK market before the end of the period starting when the UK left the EU on January 31, 2020 and ending on December 31, 2020 (the Brexit Transition Period) will be taken into account. If, after the end of the Brexit Transition Period, the drug is not placed on any other market of the remaining member states of the EU, the three-year period will start running from the last date the drug was placed on the UK market before the end of the Brexit Transition Period.

Advanced Therapy Medicinal Products

In the EU, medicinal products, including advanced therapy medicinal products ("ATMPs") are subject to extensive pre-and post-market regulation by regulatory authorities at both the EU and national levels. ATMPs comprise gene therapy products, somatic cell therapy products and tissue engineered products, which are genes, cells or tissues that have undergone substantial manipulation and that are administered to human beings in order to cure, diagnose or prevent diseases or regenerate, repair or replace a human tissue. Pursuant to Regulation (EC) No 1394/2007, the Committee for Advanced Therapies ("CAT") is responsible in conjunction with the CHMP for the evaluation of ATMPs. The CHMP and CAT are also responsible for providing guidelines on ATMPs. These guidelines provide additional guidance on the factors that the EMA will consider in relation to the development and evaluation of ATMPs and include, among other things, the preclinical studies required to characterize ATMPs. Although such guidelines are not legally binding, compliance with them is often necessary to gain and maintain approval for product candidates.

In addition to the mandatory RMP, the holder of a MA for an ATMP must put in place and maintain a system to ensure that each individual product and its starting and raw materials, including all substances coming into contact with the cells or tissues it may contain, can be traced through the sourcing, manufacturing, packaging, storage, transport and delivery to the relevant healthcare institution where the product is used.

Exceptional Circumstances/Conditional Approval

Similar to accelerated approval regulations in the United States, conditional MAs can be granted in the EU in exceptional circumstances. A conditional MA can be granted for medicinal products where, although comprehensive clinical data referring to the safety and efficacy of the medicinal product have not been supplied, a number of criteria are fulfilled: (i) the benefit/risk balance of the product is positive, (ii) it is likely that the applicant will be in a position to provide the comprehensive clinical data, (iii) unmet medical needs will be fulfilled by the grant of the MA and (iv) the benefit to public health of the immediate availability on the market of the medicinal product concerned outweighs the risk inherent in the fact that additional data are still required. Once a conditional MA has been granted, the MA holder must fulfil specific obligations within defined timelines. A conditional MA is valid for one year and must be renewed annually, but it can be converted into a standard MA once the MA holder fulfils the obligations imposed and the complete data confirm that the medicine's benefits continue to outweigh its risks.

Data and Market Exclusivity

As in the United States, it may be possible to obtain a period of market and / or data exclusivity in the EU that would have the effect of postponing the entry into the marketplace of a competitor's generic, hybrid or biosimilar product (even if the

pharmaceutical product has already received a MA) and prohibiting another applicant from relying on the MA holder's pharmacological, toxicological and clinical data in support of another MA for the purposes of submitting an application, obtaining MA or placing the product on the market. Innovative medicinal products, referred to as New Chemical Entities ("NCE"), approved in the EU qualify for eight years of data exclusivity and 10 years of marketing exclusivity.

An additional non-cumulative one-year period of marketing exclusivity is possible if during the data exclusivity period (the first eight years of the 10-year marketing exclusivity period), the MA holder obtains an authorization for one or more new therapeutic indications that are deemed to bring a significant clinical benefit compared to existing therapies.

The data exclusivity period begins on the date of the product's first MA in the EU. After eight years, a generic product application may be submitted, and generic companies may rely on the MA holder's data. However, a generic product cannot launch until two years later (or a total of 10 years after the first MA in the EU of the innovator product), or three years later (or a total of 11 years after the first MA in the EU of the innovator product) if the MA holder obtains MA for a new indication with significant clinical benefit within the eight-year data exclusivity period. Additionally, another noncumulative one-year period of data exclusivity can be added to the eight years of data exclusivity where an application is made for a new indication for a well-established substance, provided that significant pre-clinical or clinical studies were carried out in relation to the new indication. Another year of data exclusivity may be added to the eight years, where a change of classification of a pharmaceutical product has been authorized on the basis of significant pre-trial tests or clinical trials (when examining an application by another applicant for or holder of market authorization for a change of classification of the same substance the competent authority will not refer to the results of those tests or trials for one year after the initial change was authorized).

Products may not be granted data exclusivity since there is no guarantee that a product will be considered by the EU's regulatory authorities to include a NCE. Even if a compound is considered to be a NCE and the MA applicant is able to gain the prescribed period of data exclusivity, another company nevertheless could also market another version of the medicinal product if such company can complete a full MAA with their own complete database of pharmaceutical tests, preclinical studies and clinical trials and obtain MA of its product.

On April 26, 2023, the EC submitted a proposal for the reform of the European pharmaceutical legislation and negotiations are still ongoing. The timing for finalization of these negotiations and entry into force are unclear. The current drafts envisage (i) a shortening of the periods of data exclusivity from eight to six years (with transferrable vouchers for an additional year of market protection as an incentive for the development of new antibiotics), (ii) earlier regulatory guidance and extension of market exclusivity for orphan medicines (depending on certain conditions), (iii) four-year data exclusivity for additional indications of existing products, and (iv) rules governing the availability of products (including shortage prevention plans and some supply obligations for manufacturers).

Orphan Designation and Exclusivity

The criteria for designating an orphan medicinal product in the EU are similar in principle to those in the United States. The EMA grants orphan drug designation if the medicinal product is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting no more than five in 10,000 persons in the EU (prevalence criterion). In addition, Orphan Drug Designation can be granted if, for economic reasons, the medicinal product would be unlikely to be developed without incentives and if there is no other satisfactory method approved in the EU of diagnosing, preventing, or treating the condition, or if such a method exists, the proposed medicinal product is a significant benefit to patients affected by the condition. An application for orphan drug designation (which is not a MA, as not all orphan-designated medicines reach the authorization application stage) must be submitted first before an application for MA of the medicinal product is submitted. The applicant will receive a fee reduction for the MAA if the orphan drug designation has been granted, but not if the designation is still pending at the time the MA is submitted, and sponsors must submit an annual report to EMA summarizing the status of development of the medicine. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. Designated orphan medicines are eligible for conditional MA.

The EMA's Committee for Orphan Medicinal Products ("COMP") reassesses the orphan drug designation of a product in parallel with the review for a MA; for a product to benefit from market exclusivity it must maintain its orphan drug designation at the time of MA review by the EMA and approval by the EC. Additionally, any MA granted for an orphan medicinal product must only cover the therapeutic indication(s) that are covered by the orphan drug designation. Upon the grant of a MA, orphan drug designation provides up to ten years of market exclusivity in the orphan indication.

During the 10-year period of market exclusivity, with a limited number of exceptions, the regulatory authorities of the EU Member States and the EMA may not accept applications for MA, accept an application to extend an existing MA or grant a

MA for other similar medicinal products for the same therapeutic indication. A similar medicinal product is defined as a medicinal product containing a similar active substance or substances as contained in a currently authorized orphan medicinal product, and which is intended for the same therapeutic indication. An orphan medicinal product can also obtain an additional two years of market exclusivity for an orphan-designated condition when the results of specific studies are reflected in the Summary of Product Characteristics ("SmPC") addressing the pediatric population and completed in accordance with a fully compliant Pediatric Investigation Plan ("PIP"). No extension to any supplementary protection certificate can be granted on the basis of pediatric studies for orphan indications.

The 10-year market exclusivity may 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, i.e. the condition prevalence or financial returns criteria under Article 3 of Regulation (EC) No. 141/2000 on orphan medicinal products. When the period of orphan market exclusivity for an indication ends, the orphan drug designation for that indication expires as well. Orphan exclusivity runs in parallel with normal rules on data exclusivity and market protection. Additionally, a MA may be granted to a similar medicinal product (orphan or not) for the same or overlapping indication subject to certain requirements.

In the UK, following the post-Brexit transition period, a system for incentivizing the development of orphan medicines was introduced. Overall, the requirements for orphan designation largely replicate the requirements in the EU and the benefit of market exclusivity has been retained. Products with an orphan designation in the EU can be considered for an orphan MA in Great Britain and, marketing authorizations granted for products that fulfil UK orphan criteria are valid UK-wide regardless of whether there is an EU orphan designation. The MHRA will review applications for orphan designation at the time of a MA, and will offer incentives, such as market exclusivity and full or partial refunds for MA fees to encourage the development of medicines in rare diseases. Separately, the MHRA has stated that it is considering updating its licensing framework for orphan medicines, with a draft framework expected by spring 2026.

Pediatric Development
In the EU, companies developing a new medicinal product are obligated to study their product in children and must therefore submit a PIP together with a request for agreement to the EMA. The EMA issues a decision on the PIP based on an opinion of the EMA's Pediatric Committee ("PDCO"). Companies must conduct pediatric clinical trials in accordance with the PIP approved by the EMA, unless a deferral (e.g. until enough information to demonstrate its effectiveness and safety in adults is available) or waiver (e.g. because the relevant disease or condition occurs only in adults) has been granted by the EMA. The MAA for the medicinal product must include the results of all pediatric clinical trials performed and details of all information collected in compliance with the approved PIP, unless a waiver or a deferral has been granted, in which case the pediatric clinical trials may be completed at a later date. Medicinal products that are granted a MA on the basis of the pediatric clinical trials conducted in accordance with the approved PIP are eligible for a six month extension of the protection under a supplementary protection certificate (if any is in effect at the time of approval) or, in the case of orphan medicinal products, a two year extension of the orphan market exclusivity. This pediatric reward is subject to specific conditions and is not automatically available when data in compliance with the approved PIP are developed and submitted. An approved PIP is also required when a MA holder wants to add a new indication, medicinal form or route of administration for a medicine that is already authorized and covered by intellectual property rights.

In the UK, the MHRA has published guidance on the procedures for UK Paediatric Investigation Plans ("PIPs") which, where possible, mirror the submission format and requirements of the EU system. From January 1, 2025, EU pediatric requirements are addressed via Windsor Framework categorization: for Category 2 products, both UK and EU pediatric requirements apply, and an EU-agreed PIP must also be in place (unless waived).

PRIME Designation
In March 2016, the EMA launched an initiative to facilitate development of product candidates in indications, often rare, for which few or no therapies currently exist. The Priority Medicines ("PRIME") scheme is intended to encourage drug development in areas of unmet medical need and provides accelerated assessment of products representing substantial innovation reviewed under the centralized procedure. Products from small-and medium-sized enterprises may qualify for earlier entry into the PRIME scheme than larger companies on the basis of compelling non-clinical data and tolerability data from initial clinical trials. Many benefits accrue to sponsors of product candidates with PRIME designation, including but not limited to, early and proactive regulatory dialogue with the EMA, frequent discussions on clinical trial designs and other development program elements, and potentially accelerated MAA assessment once a dossier has been submitted. Importantly, once a candidate medicine has been selected for the PRIME scheme, a dedicated contact point and rapporteur from the CHMP or from CAT are appointed facilitating increased understanding of the product at EMA's Committee level. A kick-off meeting with the CHMP/CAT rapporteur initiates these relationships and includes a team of multidisciplinary experts to provide guidance on the

overall development plan and regulatory strategy. PRIME eligibility does not change the standards for product approval, and there is no assurance that any such designation or eligibility will result in expedited review or approval.

Post-Approval Regulation

Similar to the United States, both MA holders and manufacturers of medicinal products are subject to comprehensive regulatory oversight by the EMA, the EC and/or the competent regulatory authorities of the EU Member States. This oversight applies both before and after grant of manufacturing licenses and MAs. It includes control of compliance with EU good manufacturing practices rules, manufacturing authorizations, pharmacovigilance rules and requirements governing advertising, promotion, sale, and distribution, recordkeeping, importing and exporting of medicinal products.

Failure by us or by any of our third-party partners, including suppliers, manufacturers and distributors to comply with EU laws and the related national laws of individual EU Member States governing the conduct of clinical trials, manufacturing approval, MA of medicinal products and marketing of such products, both before and after grant of MA, statutory health insurance, bribery and anti-corruption or other applicable regulatory requirements may result in administrative, civil or criminal penalties. These penalties could include delays or refusal to authorize the conduct of clinical trials or to grant MA, product withdrawals and recalls, product seizures, suspension, withdrawal or variation of the MA, total or partial suspension of production, distribution, manufacturing or clinical trials, operating restrictions, injunctions, suspension of licenses, fines and criminal penalties.

The holder of a MA for a medicinal product must also comply with EU pharmacovigilance legislation and its related regulations and guidelines, which entail many requirements for conducting pharmacovigilance, or the assessment and monitoring of the safety of medicinal products.

These pharmacovigilance rules can impose on holders of MAs the obligation to conduct a labor intensive collection of data regarding the risks and benefits of marketed medicinal products and to engage in ongoing assessments of those risks and benefits, including the possible requirement to conduct additional clinical studies or post-authorization safety studies to obtain further information on a medicine's safety, or to measure the effectiveness of risk-management measures, which may be time consuming and expensive and could impact our profitability. MA holders must establish and maintain a pharmacovigilance system and appoint an individual qualified person for pharmacovigilance, who is responsible for oversight of that system. Key obligations include expedited reporting of suspected serious adverse reactions and submission of Periodic Safety Update Reports ("PSURs") in relation to medicinal products for which they hold MAs. The EMA reviews PSURs for medicinal products authorized through the centralized procedure. If the EMA has concerns that the risk benefit profile of a product has varied, it can adopt an opinion advising that the existing MA for the product be suspended, withdrawn or varied. The agency can advise that the MA holder be obliged to conduct post-authorization Phase IV safety studies. If the EC agrees with the opinion, it can adopt a decision varying the existing MA. Failure by the MA holder to fulfill the obligations for which the EC's decision provides can undermine the ongoing validity of the MA.

More generally, non-compliance with pharmacovigilance obligations can lead to the variation, suspension or withdrawal of the MA for the product or imposition of financial penalties or other enforcement measures.

The manufacturing process for pharmaceutical products in the EU is highly regulated and regulators may shut down manufacturing facilities that they believe do not comply with regulations.

Manufacturing requires a manufacturing authorization, and the manufacturing authorization holder must comply with various requirements set out in the applicable EU laws, regulations and guidance, including Directive 2001/83/EC, Directive 2003/94/EC (repealed by Directive 2017/1572 on January 31, 2022), Regulation (EC) No 726/2004 and the European Commission Guidelines for Good Manufacturing Practice. These requirements include compliance with EU GMP standards when manufacturing pharmaceutical 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. Amendments or replacements of at least Directive 2001/83/EC and Regulation (EC) No 726/2004 are part of the reform proposal for European pharmaceutical legislation. Similarly, the distribution of pharmaceutical products into and within the EU is subject to compliance with the applicable EU laws, regulations and guidelines, including the requirement to hold appropriate authorizations for distribution granted by the competent authorities of the EU Member States. The manufacturer or importer must have a qualified person who is responsible for certifying that each batch of product has been manufactured in accordance with GMP, before releasing the product for commercial distribution in the EU or for use in a clinical trial. Manufacturing facilities are subject to periodic inspections by the competent authorities for compliance with GMP.

On October 27, 2025, the Council of the EU approved a framework for compulsory licensing of crisis-relevant products (including medicinal products) in crisis situations. While the proposal focuses on voluntary agreements with intellectual property rights holders, it includes rules on compulsory licensing as a measure of last resort upon activation / declaration of a crisis or emergency mode. The European Parliament has not yet voted on the proposal.

Sales and Marketing Regulations

The advertising and promotion of our products is also subject to EU laws concerning promotion of medicinal products, interactions with physicians, misleading and comparative advertising and unfair commercial practices. In addition, other national legislation of individual EU Member States may apply to the advertising and promotion of medicinal products and may differ from one country to another. These laws require that promotional materials and advertising in relation to medicinal products comply with the product's SmPC as approved by the competent regulatory authorities. The SmPC is the document that provides information to physicians concerning the safe and effective use of the medicinal product. It forms an intrinsic and integral part of the MA granted for the medicinal product. Promotion of a medicinal product that does not comply with the SmPC is considered to constitute off-label promotion. All advertising and promotional activities for the product must be consistent with the approved SmPC and therefore all off-label promotion is prohibited. Direct-to-consumer advertising of prescription-only medicines is also prohibited in the EU. Violations of the rules governing the promotion of medicinal products in the EU could be penalized by administrative measures, fines and imprisonment. These laws may further limit or restrict the advertising and promotion of our products to the general public and may also impose limitations on its promotional activities with healthcare professionals. EU regulation with regards to dispensing, sale and purchase of medicines has generally been preserved in the UK following Brexit, through the Human Medicines Regulations. However, organizations wishing to sell medicines online need to register with the MHRA. Following Brexit, the requirements to display the common logo no longer apply to UK-based online sellers, except for those established in Northern Ireland.

Anti-Corruption Legislation

In the EU, interactions between pharmaceutical companies and physicians are also governed by strict laws, regulations, industry self-regulation codes of conduct and physicians' codes of professional conduct both at EU level and in the individual EU Member 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 prohibited in the EU. The provision of benefits or advantages to physicians is also governed by the national anti-bribery laws of the EU Member States. Violation of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain EU Member States also must be publicly disclosed. Moreover, agreements with physicians must often be the subject of prior notification and approval by the physician's employer, his/her regulatory professional organization, and/or the competent 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 individual EU Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

In the UK, the pharmaceutical sector is recognized as being particularly vulnerable to corrupt practices, some of which fall within the scope of the Bribery Act 2010. Due to the Bribery Act 2010's far-reaching territorial application, the potential penalized act does not have to occur in the UK to become within its scope. If the act or omission does not take place in the UK, but the person's act or omission would constitute an offense if carried out there and the person has a close connection with the UK, an offense will still have been committed.

The Bribery Act 2010 is comprised of four offenses that cover (i) individuals, companies and partnerships that give, promise or offer bribes, (ii) individuals, companies and partnerships that request, agree to receive or accept bribes, (iii) individuals, companies and partnerships that bribe foreign public officials and (iv) companies and partnerships that fail to prevent persons acting on their behalf from paying bribes. The penalties imposed under the Bribery Act 2010 depend on the offence committed, harm and culpability and penalties range from unlimited fines to imprisonment for a maximum term of ten years and in some cases both.

Regulations in the UK and Other Markets

The UK formally left the EU on January 31, 2020 and EU laws now only apply to the UK in respect of Northern Ireland as laid out in the protocol on Ireland and Northern Ireland and as amended by the Windsor Framework sets out a long-term set of arrangements for the supply of medicines into Northern Ireland. The EU and the UK agreed on a trade and cooperation agreement, which includes provisions affecting the life sciences sector (including on customs and tariffs). There are some specific provisions concerning pharmaceuticals, including the mutual recognition of GMP, inspections of manufacturing

facilities for medicinal products and GMP issued documents. The TCA does not, however, contain wholesale mutual recognition of UK and EU pharmaceutical regulations and product standards.

The UK government has adopted the Medicines and Medical Devices Act 2021 (the "MMDA") to enable the UK's regulatory frameworks to be updated following the UK's departure from the EU. The MMDA introduces regulation-making, delegated powers covering the fields of human medicines, clinical trials of human medicines, veterinary medicines and medical devices. The MHRA has since been consulting on future regulations for medicines and medical devices in the UK.

For other countries outside of the EU, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, again, the clinical trials must be conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

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

Additional Regulation

In addition to the foregoing, local, state and federal laws regarding such matters as safe working conditions, manufacturing practices, environmental protection, fire hazard control and hazardous substances, including the Occupational Safety and Health Act, the Resource Conservancy and Recovery Act and the Toxic Substances Control Act, affect our business. These and other laws govern our use, handling and disposal of various biological, chemical and radioactive substances used in, and wastes generated by, our operations. If our operations result in contamination of the environment or expose individuals to hazardous or biohazardous substances, we could be liable for damages, environmental remediation, and/or governmental fines. We believe that we are in material compliance with applicable environmental laws and occupational health and safety laws that continued compliance therewith will not have a material adverse effect on our business. We cannot predict, however, how changes in these laws may affect our future operations. We may incur significant costs to comply with such laws and regulations now or in the future.

Human Capital Policies and Procedures

As of December 31, 2025, we had 45 full-time employees. Of these employees, 29 were engaged in research and development and 16 were engaged in general and administrative functions. 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 metropolitan area. 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

Our website address is located at www.x4pharma.com; however, the information in, or that can be accessed through, our website is not part of or incorporated by reference into this Annual Report on Form 10-K, and any references to our website are intended to be inactive textual references only provided for convenience. We make available free of charge on our website our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K, and amendments to those reports, and other filings pursuant to Section 13(a) or 15(d) of the Exchange Act, as soon as reasonably practicable after each is electronically filed with, or furnished to, the SEC. The SEC maintains an Internet site that contains reports, proxy and information statements, and other information regarding reports that we file or furnish electronically with them at www.sec.gov.

This Annual Report on Form 10-K includes our trademarks and registered trademarks, including "XOLREMDI" Each other trademark, trade name or service mark appearing in this Annual Report on Form 10-K belongs to their respective holders.

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. The risks and uncertainties described below are not the only ones facing us. Additional risks and uncertainties not presently known to us, or that we currently see as immaterial, may also adversely affect our business. 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. The disclosures in this section reflect our beliefs and opinions as to factors that could materially and adversely affect us in the future. References to past events are provided by way of example only and are not intended to be a complete listing or a representation as to whether or not such factors have occurred in the past. The information discussed below should be considered carefully with the other information contained in this Annual Report on Form 10-K and the other documents and materials we file with the SEC, as well as news releases and other information we publicly disseminate from time to time.

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:

- Until we achieve profitability, our operations will require substantial additional funding. Our history of recurring losses and anticipated future expenditures could raise substantial doubts about our ability to continue as a going concern. 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 have incurred significant losses and have not generated significant 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.

- 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.

- Changes in estimates regarding fair value of intangible assets may result in an adverse impact on our results of operations.

- We have not generated significant revenues from product sales since inception and may never become profitable. We may never be able to generate meaningful revenues from sales of our product candidates and approved commercial products at levels or on timing necessary to support our investment and goals.

- If the commercial opportunity for mavorixafor in chronic neutropenic ("CN") 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.

- We depend almost entirely on the success of our future product candidate, mavorixafor. We cannot be certain that we will be able to obtain regulatory approval for, or successfully commercialize, mavorixafor for disorders other than WHIM, including CN, or any other product candidate.

- We may develop mavorixafor, and future product candidates, in combination with other therapies, which could expose us to additional risks.

- Our product candidates that have received regulatory approval may still face future development and regulatory difficulties and any approved products will be subject to extensive post-approval regulatory requirements. Additionally, our approved product, XOLREMDI, and future approved products, if any, 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 Food and Drug Administration ("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.

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

- If we are unable to maintain effective sales and marketing capabilities or to selectively enter into agreements with third parties to sell and market our product or product candidates, we may not be successful in commercializing our product candidates that have been approved.

- Our relationships with customers and third-party payors are 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.

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

- 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.

- We have limited experience manufacturing our product or 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.

- We rely on third-party clinical research organizations ("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 sale of our product.

- 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.

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

- If we are unable to protect our intellectual property rights, our competitive position could be harmed.
- 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.
- 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.
- 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 may experience difficulties in managing the reduction in size of our organization due to our restructuring activities, and we may not achieve the expected benefits of such activities.
- 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.
- Our internal information technology systems and infrastructure, or those of our contractors, consultants, or other third parties, may be subject to cyber attacks, or data breaches, compromises, or other security incidents, which could result in additional costs, loss of revenue, significant liabilities, harm to our reputation, and disruption of our development programs and operations.
- Although we are currently in compliance with the Nasdaq continued listing requirements, if we are unable to maintain 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.
- Our stock price has been and is likely to continue to be volatile and fluctuate substantially.
- "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.
- 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.

Risks Related to Our Financial Position and Need for Additional Capital

Until we achieve profitability, our operations will require substantial additional funding. Our history of recurring losses and anticipated future expenditures could raise substantial doubts about our ability to continue as a going concern. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate any product development programs or commercialization efforts.

Our operations have consumed a large amount of cash since inception. To date, we have funded our operations primarily with proceeds from sales of common stock, warrants, prefunded warrants, and preferred stock, proceeds from the issuance of convertible debt and borrowings under loan and security agreements. We expect to continue to incur research and development expenses 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. We expect to incur significant commercialization expenses related to product sales, marketing, distribution and manufacturing. Furthermore, we expect to continue to incur costs associated with operating as a public company.

As of December 31, 2025, we have cash and cash equivalents of $217.0 million and short-term marketable securities of $35.9 million, which provides funding for our operations into 2028. Until we become profitable, our operations will require us to obtain additional funding in the future. If we are unable to obtain sufficient funding when needed in the future, our business, prospects, financial condition and results of operations will be materially and adversely affected, and we may be unable to continue as a going concern. Such additional funding 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. If we are unable to raise capital when needed or on acceptable terms, we would be forced to delay, limit, reduce, restructure or terminate our product development or future commercialization efforts of one or more of our product candidates, or may be forced to reduce, restructure or terminate our operations. If we are unable to continue as a going concern, we may have to liquidate our assets and may receive less than the value at which those assets are carried on our audited financial statements, and it is likely that investors will lose all or a part of their investment. In our own future required quarterly assessments, we may again conclude that there is substantial doubt about our ability to continue as a going concern, and future reports from our independent registered public accounting firm may also contain statements expressing substantial doubt about our ability to continue as a going concern. If we seek additional financing to fund our business activities in the future and there exists substantial doubt about our ability to continue as a going concern, investors or other financing sources may be unwilling to provide additional funding to us on commercially reasonable terms, if at all.

Although we believe our current cash, cash equivalents and short-term marketable securities, together with our expected decrease in annual spending under our previously announced February 2025 Restructuring and September 2025 Restructuring, respectively, will be sufficient to support our operations into 2028, subject to our continued compliance with our Hercules Loan Agreement covenants, there can be no assurance that these initiatives will be successful or that any anticipated spending reductions will be adequate, which could materially affect our future operations.

While we have successfully raised capital in the past, our ability to raise capital in future periods is not assured. We may be required to raise additional capital to satisfy the cash covenant under our existing debt facility with Hercules that requires that we maintain a minimum level of cash at a level greater than 20% of our outstanding borrowings under the Hercules Loan Agreement and subject to certain operational covenants. Based on our current cash flow projections, excluding additional sources of external financing, we anticipate that we will be able to maintain the minimum cash required to satisfy this covenant for at least the next 12 month period following the issuance of these consolidated financial statements. See also the risk factor titled “Our term loan contains restrictions that limit our flexibility in operating our business” below.

We have incurred significant losses and have not generated significant 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 commercial-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, become commercially viable, or maintain commercial viability. Since inception, we have incurred significant operating losses. Our net losses were $79.2 million, $37.5 million, and $101.2 million for the years ended December 31, 2025, 2024, and 2023. We generated negative operating cash flows in each of these periods.

We expect to continue to incur significant expenses and operating losses for at least the next several years as we conduct our global, pivotal Phase 3 clinical trial for mavorixafor for the treatment of CN (the “4WARD” trial); 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; further grow a sales, marketing and distribution infrastructure to commercialize product candidates 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/or 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:

- our ability to generate revenue from mavorixafor;
- outcomes and timing of regulatory reviews, approvals and other actions;
- our ability to manufacture any approved products on commercially reasonable terms;
- our ability to maintain an effective sales and marketing organization or suitable third-party alternatives for any approved products;

- 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 4WARD Phase 3 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 market our approved product and obtain marketing approval for our product candidates;
- our ability to manage our cash expenditures in line with our budget as approved by our Board of Directors, which reflects the cost savings we anticipate from our restructuring activities executed in 2025;
- 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;
- our ability to hire additional clinical, regulatory and scientific personnel; and
- the extent of legal, accounting and other expenses that we incur to continue to operate as a public company.

Although we have obtained marketing approval for, and have begun to commercialize one of our product candidates, 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. As we have completed the development of and obtained marketing approval in the U.S. for mavorixafor, we will need to transition from a company with a research and development focus to a company capable of supporting commercial activities. We may encounter unforeseen expenses, difficulties, complications and delays, and may not be successful in such a transition.

We 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 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, including for additional indications;
- the success of our exclusive licensing and supply agreement which Norgine and any potential regulatory and commercial milestone payments that we may receive under that agreement;
- 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 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 product and licensing revenues that are in excess of our operating expense, 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. 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. 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 be required 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, restructure 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.

Changes in estimates regarding fair value of intangible assets may result in an adverse impact on our results of operations.
We test our 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 a significant impairment charge in the period that the change becomes known.

Risks Related to Development of Our Product Candidates

We have not generated significant revenues from product sales since inception and may never become profitable. We may never be able to generate meaningful revenues from sales of our product candidates and approved commercial products at levels or on timing necessary to support our investment and goals.

To date, we have not generated significant revenues from product sales and cannot predict whether or when we will be able to generate meaningful revenues from sales of our product candidates and our approved commercial products at levels or on timing necessary to support our investment and goals. Our ability to generate revenue and become profitable depends upon our ability to successfully commercialize mavorixafor. Even if we are able to successfully achieve regulatory approval for our 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 future product candidates also depends on a number of additional factors, including but not limited to our ability to:

- successfully complete development activities, including all necessary nonclinical studies and clinical trials;
- complete and submit NDAs to the FDA and obtain regulatory approval for indications for which there is a commercial market;
- complete and submit marketing applications to, and obtain regulatory approval from, foreign regulatory authorities;
- set and obtain a commercially viable price for our products;
- obtain commercial quantities of our products at acceptable cost levels;
- further 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 meaningful revenues from the sale of our product, 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.

If the commercial opportunity for mavorixafor in 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. We continue to progress our 4WARD trial to evaluate the efficacy, safety, and tolerability of oral, once-daily mavorixafor (with or without stable doses of G-CSF) in people with congenital, acquired primary autoimmune, or idiopathic CN who are experiencing recurrent and/or serious infections. We estimate that there are approximately 15,000 patients in the U.S. with CN who experience serious and/or recurring infections. We plan to initially focus on one third of these patients, representing our initial target market penetration. If the commercial opportunity 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 CN disorders. Our projections of the number of people who have CN 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. 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.

We depend almost entirely on the success of our future product candidate, mavorixafor. We cannot be certain that we will be able to obtain regulatory approval for, or successfully commercialize, mavorixafor for disorders other than WHIM, including CN, or any other product candidate.

Our business depends almost entirely on the successful clinical development, regulatory approval and commercialization of mavorixafor. We currently have only one product for sale, XOLREMDI, and may never be able to develop additional 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 additional indications for mavorixafor or any other product candidates that we may develop. We also may not be able to finalize the design or formulation for our other programs. We may not be able to complete development of any additional 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 for additional indications for 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 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 though XOLREMDI received marketing approval, 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 of, or if safety, efficacy, manufacturing or supply issues arise with, the drugs that we choose to evaluate in combination with mavorixafor or any product candidate we develop, we may be unable to obtain approval of or market mavorixafor or any product candidate we develop.

Disruptions at the FDA, the SEC and other government agencies caused by funding shortages or global health concerns, in addition to substantial uncertainty regarding the Trump administration's initiatives and staffing cuts and how these might impact the FDA, its implementation of laws, regulations, policies and guidance, and its personnel, 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, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory, and policy changes. As a result of some of these factors, average review times at the FDA have fluctuated in recent years. 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. Without appropriation of additional funding to federal agencies, our business operations related to our product development activities for the U.S. market could be impacted.

Disruptions at the FDA and other agencies, including substantial leadership departures, personnel cuts, and policy changes, 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. Changes and cuts in FDA staffing could result in delays in the FDA's responsiveness or in its ability to review IND submissions or applications, issue regulations or guidance, or implement or enforce regulatory requirements in a timely fashion or at all. For example, over the last several years the U.S. government has shut down several times, including as recently as October 2025, and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical government employees and stop critical activities. In 2025, the FDA has been subject to reductions in force and reorganization. A prolonged government shutdown or significant leadership, personnel and/or policy changes, or substantial modification in agency activities could significantly impact the ability of the FDA or other regulatory authorities to conduct their regular inspections, reviews, or other regulatory activities. Additionally, a prolonged shutdown 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, including INDs placed on clinical holds or delayed new drug approvals. 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.

With the changes being implemented by the Trump administration in 2025, there is substantial uncertainty as to whether and how the Trump administration will seek to modify or revise the requirements and policies of the FDA and other regulatory agencies with jurisdiction over our product candidates and any products for which we obtain approval. This uncertainty could present new challenges and/or opportunities as we navigate development and approval of our product candidates. Some of these efforts have manifested to date in the form of personnel cuts and measures that could impact the FDA's ability to hire and retain key personnel, which could result in delays or limitations on our ability to obtain guidance from the FDA on our product candidates in development and obtain the requisite regulatory approvals in the future. There remains general uncertainty regarding future activities. The Trump administration could issue or promulgate executive orders, regulations, policies or guidance that adversely affect us or create a more challenging or costly environment to pursue the development of new therapeutic products. Alternatively, state governments may attempt to address or react to changes at the federal level with changes to their own regulatory frameworks in a manner that is adverse to our operations. If we become negatively impacted by future governmental orders, regulations, policies or guidance as a result of the Trump administration, there could be a material adverse effect on us and our business.

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 additional indications for 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 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 future NDA submissions 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 other indications for many reasons, including, among others:

- disagreement with the design or implementation and sufficiency of our clinical trials;
- failure to demonstrate the safety and efficacy of mavorixafor or any other product candidate for its proposed indications and that;
- any clinical and other benefits of mavorixafor or any other product candidate outweigh its safety risks;
- 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. If our current or future product candidates receive regulatory approval, these 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 and others 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 and other entities 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 obligation to pay Genzyme (i) future milestone payments in the aggregate amount of up to $10.0 million as of December 31, 2025, contingent upon our achievement of certain late-stage regulatory and sales milestones with respect to licensed products (ii) our obligation to pay Genzyme tiered royalties based on net sales of licensed products that we commercialize under the agreement; and (iii) 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 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.

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 or 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 registration 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 3 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 WHIM syndrome. 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 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 public health emergencies and geopolitical conflicts such as the war in Ukraine or in Gaza; 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. For XOLREMDI and any other product candidates that receive marketing approval in the future, if 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 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 or product candidates and could substantially increase the costs of commercializing our products or product candidates, and significantly impact our ability to successfully commercialize our products or 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.

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 patients. 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.

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

Our product candidates that have received regulatory approval may still face future development and regulatory difficulties and any approved products will be subject to extensive post-approval regulatory requirements. Additionally, our approved product, XOLREMDI, and future approved products, if any, could be subject to marketing restrictions or withdrawal from the market, and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products.

Any product candidate that receives regulatory approval will 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"), impose significant restrictions on our product's indicated uses or marketing, or 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.

Our approved product, XOLREMDI, and future product candidates that receive marketing approval, if any, 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. The marketing approval of our product candidate 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 our 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 or product candidates or the manufacturing facilities for our product or 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 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 or imposed permanent injunctions under which specified promotional conduct is changed or curtailed. If we cannot successfully manage the promotion of our products, we could become subject to significant liability, which would materially adversely affect our business and financial condition.

Our commercial success depends upon attaining significant market acceptance of our approved product or product candidates, if approved, among hospitals, physicians, patients and healthcare payors.
Our approved product may not gain market acceptance among hospitals, physicians, health care payors, patients and the medical community. Market acceptance of any of our approved product or product candidates for which we receive approval in the future, if any, 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 products and product candidates over alternative treatments as compared to their relative costs;
- the prevalence and severity of any side effects with respect to our products or 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 products or product candidates on hospital or insurance formularies or limitations on coverages that may be available in the early stages of commercialization for newly approved drugs. If our product or any product candidate that is approved fails 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 we are unable to maintain effective sales and marketing capabilities or to selectively enter into agreements with third parties to sell and market our product or product candidates, we may not be successful in commercializing our product candidates that have been approved.
To achieve commercial success for our approved product for which we retain sales and marketing responsibilities, we must either build or outsource a focused sales and marketing infrastructure to sell our approved product. If we are unable to establish

and maintain 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, such as the exclusive licensing and supply agreement we entered into with Norgine in January 2025. 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 revenues 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 or 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 or 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.

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 though we have obtained FDA approval for one of our product candidates, 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 candidate 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 European Medicines Agency ("EMA") could 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. If marketing authorization for mavorixafor in the European Union is granted under exceptional circumstances, such authorization would be subject to specific post-authorization obligations and annual review, and failure to comply with such obligations or adverse findings in required follow-up studies could result in modification, suspension or withdrawal of the authorization. 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.

Significant political, trade, regulatory developments, economic downturns, inflation, increases in interest rates, natural disasters, public health crises, geopolitical events, such as the war in Ukraine and in Gaza and other circumstances beyond our control, could have a material adverse effect on our business, financial condition or results of operations. Significant political, trade, or regulatory developments, such as those stemming from the change in U.S. federal administration, are difficult to predict and may have a material adverse effect on us. Similarly, changes in U.S. federal policy that affect the geopolitical landscape could give rise to circumstances outside our control that could have negative impacts on our business operations. In 2017, the U.S. Congress and the Trump administration made substantial changes to U.S. policies, which included comprehensive corporate and individual tax reform. In addition, the Trump administration called for significant changes to U.S. trade, healthcare, immigration and government regulatory policy. With the transition to the Biden administration in early 2021, changes to U.S. policy occurred and since the start of the Trump administration in 2025, U.S. policy changes have been implemented at a rapid pace and additional changes are likely. Changes to U.S. policy implemented by the U.S. Congress, the Trump administration or any new administration have impacted and may in the future impact, among other things, the U.S. and global economy, international trade relations, unemployment, immigration, healthcare, taxation, the U.S. regulatory environment, inflation and other areas. For example, in early 2025, the United States imposed blanket 10% tariffs on virtually all imports to the U.S. and significantly higher so-called reciprocal tariffs applicable to imports from many countries. In September 2025, President Trump announced 100% tariffs on imported branded or patented pharmaceuticals, effective October 1, 2025, unless the importing company is building U.S. manufacturing capacity. It is not yet clear whether these tariffs would apply to the importation of active pharmaceutical ingredients and possibly bulk drug products that are intended for use in clinical trials and not for commercial sale, which could increase the costs of materials for our clinical trials. The Trump administration has threatened to continue to broadly impose tariffs, which could lead to corresponding punitive actions by the countries with which the U.S. trades. While certain tariffs have been suspended, modified or temporarily reduced, we cannot predict the results of the U.S. government's trade negotiations or the outcome of ongoing legal challenges to specific tariff policies. Historically, tariffs have led to increased trade and political tensions. In response to tariffs, other countries have implemented retaliatory tariffs on U.S. goods. Political tensions as a result of trade policies could reduce trade volume, investment, technological exchange and other economic activities between major international economies, resulting in a material adverse effect on global economic conditions and the stability of global financial markets. Any changes in political, trade, regulatory, and economic conditions, including U.S. trade policies, could have a material adverse effect on our financial condition or results of operations. Until we know what policy changes are made, whether those policy changes are challenged and subsequently upheld by the court system and how those changes impact our business and the business of our competitors over the long term, we will not know if, overall, we will benefit from them or be negatively affected by them.

Further, the global economy, including credit and financial markets, outside of recent tariffs, 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.

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 or our collaborators may not seek, or may seek but never receive, regulatory approval to market our products, including XOLREMDI, or product candidates outside of the U.S. or in any particular country or region. In order to market any product outside of the U.S., we or our collaborators must establish and comply with the numerous and varying safety, efficacy and other regulatory requirements of other countries. Approval procedures vary among countries and can involve additional non-clinical studies or clinical trials, additional work related to manufacturing and analytical testing on controls, and additional administrative review periods. The time required to obtain approvals in other countries might differ from that required to obtain FDA approval. Marketing approval in one country does not ensure marketing approval in another, but a failure or delay in obtaining marketing approval in one country may have a negative effect on the regulatory process in other countries. The marketing approval processes in other countries may implicate all of the risks detailed above regarding FDA approval in the U.S. as well as other risks. In particular, in many countries outside of the U.S., products must receive pricing and reimbursement approval before the product can be commercialized. Obtaining this approval may require additional studies and data, and can result in substantial delays in bringing products to market in such countries and such investment may not be justified from a business standpoint given the market opportunity or level of required investment. Even if we or our collaborators generate the data and information which we or our collaborators believe may be sufficient to file an application for regulatory approval of any of our products or product candidates in a region or country outside the U.S., the relevant regulatory agency may find that we or our collaborators did not meet the requirements for approval, or even if our application is approved, we may have significant post-approval obligations.

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. Any setback or delay in obtaining regulatory approval or commencing marketing, if approved, for our product candidates in a country or region outside the U.S. where we or our collaborators have decided it makes business sense to proceed may have a material adverse effect on our business and prospects.

Any products that we commercialize 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 be subject to price regulations that delay our commercial launch of a 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 mavorixafor or future product candidates, even if those candidates obtain marketing approval.

Our ability to commercialize mavorixafor or future product candidates successfully depends in part on the extent to which coverage and adequate reimbursement for these products and related treatments are 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 mavorixafor or any future product candidate for which we obtain marketing approval. Even if favorable coverage and reimbursement status is attained, 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 XOLREMDI or for any future approved product candidates could have a material adverse effect on our operating results, our ability to raise capital needed to develop additional 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 patients;
- significant costs to defend any related litigation;
- substantial monetary awards to trial patients 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 are 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.

We have an approved, commercialized product, and we are 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, market, sell and distribute XOLREMDI or any products candidates for which we obtain marketing approval in the future. 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, 2025 titled “Business – Government Regulation – Other Healthcare Laws and Compliance Requirements.”

Efforts to ensure that our business arrangements with third parties comply with applicable healthcare laws and regulations 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 or 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 product candidates, restrict post-approval activities and affect our ability to sell profitably any approved product or product candidates for which we obtain marketing approval in the future.

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 may receive for any approved product. These 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.

Multiple recent executive actions have signaled the federal government's increasing focus on lowering prescription drug prices, adding to the uncertainty surrounding future drug pricing and reimbursement frameworks, including the executive orders titled "Delivering Most-Favored-Nation Prescription Drug Pricing," which directed the Secretary of Health and Human Services (HHS) to identify and communicate most-favored-nation price targets for prescription drugs and to propose a rulemaking plan to impose such pricing if "significant progress" is not made, and "Lowering Drug Prices by Once Again Putting Americans First," which contained a broad set of directives aimed at reducing drug costs. Separately, prior to the enactment of "An Act to provide for reconciliation pursuant to title II of H. Con. Res. 14" (the "Act"), orphan drugs were exempt from Medicare price negotiation only if they had received a single orphan designation and were approved solely for the corresponding rare disease or condition. The Act amended this exemption to apply more broadly: now, any orphan-designated drug is exempt from price negotiation, regardless of the number of orphan designations it has received, provided the drug's approved indications are exclusively for those rare diseases.

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 our operations may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval for our future product candidates, as well as subject us to more stringent product labeling and post-marketing conditions and other requirements. Moreover, the U.S. Supreme Court's June 2024 decision to overturn established case law giving deference to regulatory agencies' interpretations of ambiguous statutory language has introduced uncertainty regarding the extent to which the U.S. government regulations, policies and decisions may become subject to increasing legal challenges, delays, and/or changes.

See the sections of this Annual Report on Form 10-K for the fiscal year ended December 31, 2025 titled, "Business – Government Regulation – Coverage 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.

Risks Related to Our Dependence on Third Parties

We have limited experience manufacturing our product or 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 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 or 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 or product candidates 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. Manufacturers and other parties in the supply chain also must meet chain of distribution requirements and build electronic, interoperable systems for product tracking and tracing and for notifying the FDA of counterfeit, diverted, stolen and intentionally adulterated products or other products that are otherwise unfit for distribution in the United States. 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 or 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 limited 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 sale of our product.
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 or 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, for example the Norgine Agreement and our agreement with Taiba Middle East Fz LLC.

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 products or 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 products or 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 management team. 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.

The recent restructuring changes in our business may make it more difficult to attract and retain qualified personnel. We cannot guarantee we will achieve our goals or that our actions will result in expected benefits to our business.

We may experience difficulties in managing the reduction in size of our organization due to our restructuring activities, and we may not achieve the expected benefits of such activities.
In September 2025, we announced a strategic restructuring of our business operations, workforce and capital spending (the "September 2025 Restructuring") to sharpen operational focus and align resources with the Company's long-term strategy to successfully complete the 4WARD Phase 3 trial in patients with moderate and severe CN. As part of the September 2025 Restructuring, we implemented a net reduction of our employee headcount by approximately 50%. The workforce reduction was substantially completed in the third quarter of 2025. In addition, as part of a prior strategic restructuring plan announced in February 2025 (the "February 2025 Restructuring"), we previously implemented a net reduction of our employee headcount by 43 employees, or approximately 30% of our total workforce as of February 2025. The strategic restructuring activities included (i) discontinuing of certain research efforts, (ii) closing the Company's facility in Vienna, Austria, (iii) pausing certain pre-clinical drug candidate programs, (iv) scaling back the U.S. commercial field team and supporting roles across the Company and (v) streamlining other spending to support the ongoing clinical development of mavorixafor for the larger population of those with CN.

We may not realize, in full or in part, the anticipated benefits, savings and improvements from the September 2025

Restructuring and February 2025 Restructuring to the extent or as quickly as anticipated due to unforeseen difficulties, delays or unexpected costs. If we are unable to realize the expected operational efficiencies and cost savings from the restructuring, our operating results and financial condition could be adversely affected. As we pause our development and scale our commercialization plans and strategies back, we may lack adequate managerial, operational, development, sales, marketing, financial and other resources. Our management, personnel and systems currently in place may not be adequate to support future operations.

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.

Our internal information technology systems and infrastructure, or those of our contractors, consultants, or other third parties, may be subject to cyber attacks, or data breaches, compromises, or other security incidents, which could result in additional costs, loss of revenue, significant liabilities, harm to our reputation, and disruption of our development programs and operations.

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. Like other companies in our industry, we, and our third-party vendors, have experienced, and will continue to experience, cybersecurity threats and incidents relating to our information technology systems and infrastructure.

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 our development and commercialization efforts could be delayed and we could be required to notify impacted stakeholders (including affected individuals, regulators and investors) what could lead to significant liability through litigation and regulatory investigations and enforcement actions, including under state (e.g., state breach notification and consumer protection laws), federal (e.g., HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 ("HITECH")), and international law (e.g., the GDPR).

Our contracts may not contain limitations of liability, and even where they do, there can be no assurance that limitations of liability in our contracts are sufficient to protect us from liabilities, damages, or claims related to our privacy and data security obligations. Further, although we maintain cyber liability insurance, this insurance may not provide adequate coverage against potential liabilities related to any experienced cybersecurity incident or data breach.

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, 2025, we had U.S. federal and state NOLs of $432.0 million and $383.9 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 ("Section 382") contains rules that limit the ability of a company that undergoes an ownership change to utilize its net operating losses ("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 completed a Section 382 study that has identified ownership changes that will limit the future use of our NOL carryforwards. See Note 17 of our Annual Report on Form 10-K for a further discussion of these limitations. 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.

We may be subject to adverse legislative or regulatory changes in tax laws that could negatively impact our financial condition.
The rules dealing with U.S. federal, state and local income taxation are constantly under review by persons involved in the legislative process and by the U.S. Internal Revenue Service and the U.S. Treasury Department. For example, the One Big Beautiful Bill Act ("OBBBA") was signed into law on July 4, 2025 and made significant changes to U.S. federal tax law. Changes to tax laws (which changes may have retroactive application) could adversely affect our stockholders or us. In recent years, many changes to tax laws have been made and changes are likely to occur in the future. We cannot predict whether, when, in what form, or with what effective dates, tax laws, regulations and rulings may be enacted, promulgated or decided, which could result in an increase in our, or our stockholders' tax liability or require changes in the manner in which we operate in order to minimize increases in our tax liability.

Our term loan contains restrictions that limit our flexibility in operating our business.
Our Hercules Loan Agreement is 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.

As noted above, the Hercules Loan Agreement also requires that we maintain a minimum level of cash of greater than 20% of our outstanding borrowings under the Hercules Loan Agreement and subject to certain operational covenants.

Risks Related to Ownership of Our Common Stock

Although we are currently in compliance with the Nasdaq continued listing requirements, if we are unable to maintain 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.

Our common stock is listed on The Nasdaq Capital Market ("Nasdaq"). There are a number of continued listing requirements that we must satisfy in order to maintain our listing on Nasdaq, including the requirement to maintain a minimum bid price of at least $1.00 (the "Bid Price Rule"). If a deficiency with respect to this requirement continues for a period of 30 consecutive business days, Nasdaq may require us to satisfy a minimum bid price per share of our common stock of at least $1.00 for a period in excess of ten consecutive business days, but generally no more than 20 consecutive business days, before determining that we have demonstrated an ability to maintain long-term compliance with the Bid Price Rule. We have been unable to comply with the Bid Price Rule in the past, such as in 2025, and our continued listing on Nasdaq required the grant of a grace period from Nasdaq and the implementation of a reverse stock split. If we fail to comply with the Bid Price Rule in the future, there can be no assurance that we will be granted such grace periods or that we will be able to receive the necessary shareholder approval to implement an additional reverse stock split. In particular, we may encounter difficulties obtaining such shareholder approval due to our heavily retail investor shareholder base, which may also affect our ability to obtain shareholder approval of other significant corporate actions.

Any delisting of our common stock would likely adversely affect the market liquidity and market price of our common stock and our ability to obtain financing for the continuation of our operations and/or result in the loss of confidence by investors. If we fail to meet the continued listing requirements of Nasdaq, we could face significant material adverse consequences, including: (1) a limited availability of market quotations for our securities; (2) reduced liquidity with respect to our securities; (3) a determination that our shares are a "penny stock" if they are not already determined to be a "penny stock" at the time of such failure to meet such requirements, which will require brokers trading in our securities to adhere to more stringent rules, possibly resulting in a reduced level of trading activity in the secondary trading market for our securities; (4) a limited amount of news and analyst coverage for us; and (5) a decreased ability to issue additional securities or obtain additional financing in the future.

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. 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.

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, 2025. 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.

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 (the "Board") 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. We engage third party advisors, including assessors and cybersecurity consultants, to assess, validate and enhance our cybersecurity program. 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 and a vendor security questionnaire.

Governance Related to Cybersecurity Risks

Our Director of Infrastructure, Operations and Cybersecurity ("IT Director"), who reports to our Chief Financial Officer, has over ten years of experience managing organizational operations, security and infrastructure. The IT Director manages the Company's cybersecurity program and is responsible for the day-to-day monitoring and remediation of cybersecurity risks. The IT Director meets periodically with members of our finance and legal departments to discuss the Company's ongoing cybersecurity efforts.

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.

Since the beginning of fiscal year 2025, we have not identified risks from known cybersecurity threats or incidents that have materially affected us or are reasonably likely to materially affect us. Although risks from cybersecurity threats have to date not materially affected us, our business strategy, results of operations or financial condition this fiscal year, 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.

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 $96 thousand as of December 31, 2025, 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.

Item 3. Legal Proceedings

From time to time, we may become involved in legal proceedings or be subject to claims arising in the ordinary course of our business. We are not currently a party to any material legal proceedings and we are not aware of any pending or threatened legal proceedings against us that we believe could have a material adverse effect on our business, operating results or financial condition.

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 is traded on the Nasdaq Capital Market under the symbol "XFOR".

Holders of Our Common Stock
As of March 12, 2026, based on information from our transfer agent, there were 38 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. The actual number of stockholders is greater than this number of record holders, and includes stockholders who are beneficial owners, but whose shares are held in street name by brokers and other nominees. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

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

The following discussion and analysis is intended to provide material information around events and uncertainties known to management that are relevant to an assessment of the financial condition and results of operations of X4 Pharmaceuticals and should therefore be read in conjunction with our audited Consolidated Financial Statements and the related notes thereto and other disclosures included as part of this Annual Report on Form 10-K, including the disclosures under Part I, Item 1A. Risk Factors.

Overview

We are a biopharmaceutical company developing, and commercializing novel therapeutics for the treatment of rare hematology diseases. We continue to progress our global, pivotal Phase 3 clinical trial, (the "4WARD" trial) to evaluate the efficacy, safety, and tolerability of oral, once-daily mavorixafor (with or without stable doses of granulocyte colony-stimulating factor ("G-CSF") in people with congenital, acquired primary autoimmune, or idiopathic chronic neutropenia ("CN") who are experiencing recurrent and/or serious infections. The 52-week trial is a randomized, double-blind, placebo-controlled, multicenter study aiming to enroll up to 176 patients, with full enrollment expected in the third quarter of 2026. The U.S. Food and Drug Administration ("FDA") has granted Fast Track designation to mavorixafor for the treatment of CN, which 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). CN 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.

We have one commercially approved product, XOLREMDI ® (mavorixafor), which has received accelerated approval in the United States from the FDA for use as an oral, once-daily therapy in patients 12 years of age and older with WHIM (warts, hypogammaglobulinemia, infections, and myelokathexis) syndrome, to increase the number of circulating mature neutrophils and lymphocytes. WHIM syndrome is a rare combined primary immunodeficiency and CN disorder. In connection with our long term strategy to successfully complete the 4WARD Phase 3 trial in patients with moderate and severe CN, we are no longer prioritizing investment in the WHIM indication.

Private Placement Financing and Management Changes

During the third quarter of 2025, we sold shares of common stock and pre-funded warrants to purchase shares of common stock in a private placement that resulted in net proceeds of approximately $81.0 million, after deducting placement agent fees and other expenses. Pursuant to registration rights agreements, we registered the shares of common stock issued and issuable under pre-funded warrants under a registration statement on Form S-3 that was declared effective by the SEC on September 17, 2025.

Concurrent with the financing and effective August 12, 2025, our former President and Chief Executive Officer, Paula Ragan, PhD, and Chief Financial Officer, Adam Mostafa, stepped down from their respective roles and their employment was terminated. Dr. Ragan also resigned from the Company's Board of Directors (the "Board"), and Michael Wyzga transitioned from Board Chair to Lead Independent Director. The Board appointed Adam R. Craig, M.D., Ph.D, MBA as Executive Chairman, John Volpone as President and subsequently as Chief Operating Officer, and David Kirske as Chief Financial Officer.

Q4 2025 Equity Financing

In October 2025, we closed an underwritten public offering of our common stock and, in lieu of common stock to certain investors, pre-funded warrants to purchase shares of common stock, raising net proceeds of $145.6 million, net of underwriting discounts and estimated offering expenses.

Strategic Restructurings

- In the first quarter of 2025, we implemented a strategic restructuring of our business operations, workforce and capital spending to focus efforts on advancing mavorixafor to treat patients with CN. As part of this restructuring, we (i) implemented a net reduction of our employee headcount by 43 employees, representing approximately 30% of our total workforce, including our U.S. commercial field team, (ii) commenced the closure of our research and development facility in Vienna, Austria, (iii) paused our pre-clinical drug candidate programs and (iv) streamlined other spending to support the ongoing clinical development of mavorixafor for the larger population of those with chronic neutropenia. We incurred charges of approximately $2.1 million for severance and other employee termination-related costs related to this strategic restructuring.

- In the third quarter 2025, we announced an additional strategic restructuring designed to further sharpen operational focus and align resources with our long-term strategy to successfully complete the 4WARD Phase 3 trial in patients with moderate and severe CN. As part of this initiative, we further reduced our workforce by approximately 50%. We incurred expenses of approximately $4.9 million during the third quarter for severance and other employee termination-related costs related to this strategic restructuring. This workforce reduction was substantially completed in the third quarter of 2025.

These strategic restructuring actions have allowed us to decrease our operating expenses, including research and development and general and administrative expenses, from $143.2 million in 2024 to $116.2 million in 2025. The estimate of costs that we expect to incur related to these workforce reduction as well as the decrease in spending, and the timing thereof are subject to a number of assumptions and actual results may differ. We may also incur additional costs not currently contemplated due to events that may occur as a result of, or that are associated with, the actions described above.

Regulatory Update and Out-License Agreements

In early 2025, we submitted a Marketing Authorization Application ("MAA") to the EMA seeking regulatory approval to commercialize mavorixafor for WHIM syndrome in the European Union. Such MAA was validated for processing by the EMA in January 2025. In February 2026, the EMA's Committee for Medicinal Products for Human Use adopted a positive opinion recommending the grant of marketing authorization, under exceptional circumstances, for mavorixafor for the treatment of WHIM syndrome in the European Union. The positive opinion has been submitted to the European Commission for review, and we expect the European Commission to issue a final approval decision in the second quarter of 2026. On January 13, 2025, we announced a License and Supply Agreement (the "Norgine Agreement") with Norgine Pharma UK ("Norgine"), pursuant to which Norgine was granted an exclusive license to distribute, market and sell our drug product for all indications in the European Economic Area, Switzerland, the United Kingdom, Australia, and New Zealand.

Results of Operations

The following table summarizes the results of our operations for the years ended December 31, 2025 and 2024:

(in millions)	Year Ended December 31, 2025	2024	Change
Revenue	$ 35.1	$ 2.6	$ 32.5
Cost and operating expenses:			
Cost of revenue	5.8	0.8	5.0
Research and development	72.7	81.6	(8.9)
General and administrative	43.5	61.6	(18.1)
Gain on sale of non-financial asset	—	(105.0)	105.0
Total operating expenses	122.0	39.0	83.0
Loss from operations	(86.9)	(36.4)	(50.5)
Total other income (expense), net	7.7	(0.7)	8.4
Loss before income taxes	(79.2)	(37.1)	(42.1)
Provision for income taxes	—	(0.4)	0.4
Net loss	$ (79.2)	$ (37.5)	$ (41.7)

Revenue

License and Other

In January 2025, we granted an exclusive license to Norgine to distribute, market and sell our product for all indications in the European Economic Area, Switzerland, the United Kingdom, Australia and New Zealand following regulatory approval. For the year ended December 31, 2025, we recognized $27.6 million for the delivery of the license and $1.0 million for the provision of research and development services to Norgine. We had no license or other revenue during the year ended December 31, 2024.

Product Revenue, Net

We began recognizing product sales in June 2024 following FDA approval of XOLREMDI on April 29, 2024 and its subsequent commercial launch in the United States. Net product sales were as follows for years ended December 31, 2025 and 2024, and for each of the quarterly periods therein.

(in thousands)	2025	2024
First quarter	$ 942	$ —
Second quarter	1,744	563
Third quarter	1,566	560
Fourth quarter	2,271	1,434
Year ended December 31,	$ 6,523	$ 2,557

Co-pay assistance payments and rebates to U.S. government payors have comprised the majority of our gross-to-net revenue adjustments. Gross-to-net adjustments were approximately 10% and 9% for the years ended December 31, 2025 and 2024, respectively.

Operating Cost and Expenses:

Cost of Revenue

Cost of revenue primarily consists of amortization of an intangible asset related to accrued and paid milestone payments associated with our license agreement (the "Genzyme Agreement") with Genzyme Corporation ("Genzyme"), a wholly owned subsidiary of Sanofi, and sales and sublicense-based royalty payments due thereunder. Cost of revenue increased $5.0 million in the year ended December 31, 2025, as compared to the prior year, primarily due to additional royalties in the current year associated with sublicense income from our Norgine Agreement.

Research and Development Expenses

Research and development expenses consist primarily of costs incurred in connection with the development of our product candidates, including employee salaries and related expenses, clinical development expenses, internal and third-party costs of manufacturing our drug products for use in our clinical trials. Research and development expenses also include costs related to compliance with regulatory requirements; and prior to the FDA's approval of XOLREMDI in the U.S. in the second quarter of 2024, payments made under third-party licensing agreements were charged to research and development expense.

Following our strategic restructuring announced during the first quarter of 2025, substantially all of our research and development has been focused on our one product candidate, mavorixafor. The following table shows external costs incurred by product candidate (primarily external contract research organization costs) and unallocated research and development costs, primarily consisting of employee salaries and related expense for our research and development organization.

	Year Ended December 31,		
(in millions)	2025	2024	Change
Direct research and development expenses by product candidate:			
Mavorixafor	$ 41.4	$ 41.5	$ (0.1)
X4P-002	—	0.2	(0.2)
X4P-003	—	0.2	(0.2)
Unallocated expense	31.3	39.7	(8.4)
Total research and development expenses	$ 72.7	$ 81.6	$ (8.9)

Research and development expenses decreased by $8.9 million in the year ended December 31, 2025 as compared to the prior year primarily due to decreases in spending associated with our 2025 strategic restructurings, including lower spending on non-clinical programs, lower consulting fees, lower drug substance manufacturing costs, and lower regulatory costs. For the year ended December 31, 2025, unallocated expense includes $2.0 million in severance charges for terminated employees. The overall decrease in research and development expenses in the current year was partially offset by higher clinical costs associated with our 4WARD trial.

General and Administrative Expenses

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

General and administrative expenses decreased by approximately $18.1 million in the year ended December 31, 2025 as compared to the prior year. General and administrative expenses include $5.0 million in severance charges for terminated employees in the year ended December 31, 2025. The decrease to general and administrative expenses was primarily due to an $8.0 million decrease in compensation expense due to lower head count in our sales, general and administrative functions, and a decrease of $9.0 million in sales and marketing expenses in the current year as compared to the prior year during which we incurred commercialization sales and marketing launch costs related to XOLREMDI. These decreases in general and administrative expenses were partially offset by higher severance costs associated with our 2025 strategic restructurings and higher legal costs in the current year.

Other Income (Expense), Net

	Year Ended December 31,		
	2025	2024	change
(in millions)			
Interest income	$ 4.6	$ 5.8	$ (1.2)
Interest expense	(8.9)	(8.8)	(0.1)
Change in fair value of Class C warrant liability	12.8	1.9	10.9
Other (expense) income, net	(0.8)	0.4	(1.2)
Total other income (expense), net	$ 7.7	$ (0.7)	$ 8.4

Other income (expense), net, increased approximately $8.4 million in the year ended December 31, 2025 as compared to the prior year primarily due to higher gains in the current year on fair value adjustments related to our Class C warrants, partially offset by lower interest income earned on our marketable security investment portfolio.

Provision for Income Taxes

Our income tax provision of $41.0 thousand for the year ended December 31, 2025, which was primarily related to our Austrian subsidiary, was lower than our income tax provision in the prior year. Our income tax provision for the year ended December 31, 2024 of $0.3 million reflected U.S. federal and state taxable income that included the sale of a priority review voucher, generating $105.0 million of taxable income, partially offset by available deductions, net operating loss carryforwards, which were limited under IRC 382 due to several qualifying ownership changes, and available research and development credits. We will continue to maintain a full valuation allowance against net deferred tax assets, including net operating loss carryforwards, until we are able to consistently generate sufficient taxable income to realize the benefit of our net deferred tax assets.

Liquidity and Capital Resources

Sources of Liquidity

To date, we have funded our operations primarily with proceeds from sales of common stock, warrants, prefunded warrants and preferred stock, proceeds from the issuance of convertible debt and borrowings under loan and security agreements.

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. Most recently in August 2025, we sold shares of common stock and, in lieu of common stock to certain investors, pre-funded warrants to purchase shares of common stock in a private placement offering (the "Q3 2025 PIPE") resulting in net proceeds of $81.0 million, after deducting placement agent fees and offering expenses. In addition, in Q4 2025 we completed an underwritten public offering of 52,844,000 shares of our common stock (inclusive of 6,984,000 shares pursuant to the exercise in full of the underwriters' option to purchase additional shares) at a public offering price of $2.90 per share and, in lieu of

common stock to certain investors, prefunded warrants to purchase up to 700,000 shares of our common stock at a price of $2.899 per pre-funded warrant, for net proceeds of $145.6 million, after underwriting discounts and offering expenses.

ATM Sales Agreement. We are party to a Controlled Equity Offering℠ Sales Agreement ("ATM"), dated as of August 7, 2020, pursuant to which we may offer and sell shares of our common stock through one or more investment banks. To date, for the year ended December 31, 2025 and for the three months ended December 31, 2025, we have sold $24.2 million, $9.7 million, and $0.7 million, respectively, of our common stock, net of offering costs, under the ATM. Pursuant to our Registration Statement on Form S-3 that became effective on August 24, 2023 and the related ATM prospectus contained therein, we may offer and sell shares of our common stock having an aggregate offering price of up to an additional $65.3 million.

Product Sales and License Revenue. We commercially launched XOLREMDI in the second quarter of 2024 following the approval of XOLREMDI by the FDA on April 29, 2024. To date, we have generated $9.1 million of net product revenue from the sale of XOLREMDI. For the year ended December 31, 2025, we generated $27.6 million in license revenue from the exclusive licensing and supply agreement with Norgine.

Hercules Loan Agreement. We are a party to a loan and security agreement (the "Hercules Loan Agreement"), which provides for a term loan facility of up to $107.5 million, under which we have borrowed an aggregate of $75.0 million of term loans to date, representing the maximum borrowings as of December 31, 2025. The term loan facility requires that we make interest-only payments through maturity on July 1, 2027 and requires that we meet certain operational and financial covenants. See Note 11 to the consolidated financial statements contained herein for a full description of our Hercules Loan Agreement.

Historical Cash Flows

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

	Year Ended December 31,	
	2025	2024
	(in millions)	
Net loss	$ (79.2)	$ (37.5)
Adjustments to reconcile net loss to net cash used in operating activities	(3.8)	(96.1)
Changes in operating assets and liabilities	(2.6)	2.7
Net cash used in operating activities	(85.6)	(130.9)
Net cash provided by investing activities	8.2	67.0
Net cash provided by financing activities	238.6	20.3
Effect of exchange rate changes on cash, cash equivalents and restricted cash	0.2	(0.1)
Net increase (decrease) in cash, cash equivalents and restricted cash	161.4	(43.7)
Cash, cash equivalents and restricted cash, beginning of period	56.5	100.2
Cash, cash equivalents and restricted cash, end of period	$ 217.9	$ 56.5

Operating Activities

During the year ended December 31, 2025, net cash used in operating activities was $85.6 million, primarily resulting from net losses of $79.2 million adjusted for net non-cash income of $3.8 million, primarily related to gains on changes to the fair value of our Class C warrants that are measured quarterly at fair value, and $2.6 million of changes to operating assets and liabilities primarily related to a reduction in accounts payable and accrued expenses. Net cash used in operating activities for the year ended December 31, 2024 was $130.9 million, primarily resulting from our operating losses of $142.5 million adjusted for noncash expenses of $8.9 million and changes in our operating assets and liabilities of $2.7 million. Noncash expenses primarily include stock-based compensation expense of $8.2 million and non-cash lease expense of $1.6 million, partially offset by $1.9 million of non-cash gains on the change in fair value of our Class C Warrant liability.

Investing Activities

During the year ended December 31, 2025, cash provided by investing activities of $8.2 million primarily includes net sales of short-term marketable securities. During the year ended December 31, 2024, cash provided by investing activities was $67.0 million, primarily due to the receipt of $105.0 million of cash from the sale of a PRV, partially offset by net investments in short-term marketable securities.

Financing Activities

During the year ended December 31, 2025, cash provided by financing activities of $238.6 million was primarily due to net proceeds of $226.6 million from the sale our common stock and pre-funded warrants in private placement and public offerings and $11.9 million from sales of our common stock through our ATM program and purchase agreement with Lincoln Park Capital Fund LLC. Cash provided by financing activities for the year ended December 31, 2024 included $20.0 million of new borrowings on our loan facility.

Capital Resources

Based on our cash, cash equivalents and marketable securities on hand as of March 17, 2026, and our current operating plan, we believe that our cash, cash equivalents and marketable securities will allow us to fund operations for at least the next 12 months.

Capital Requirements

During the year ended December 31, 2025, we sold shares of common stock and, in lieu of common stock to certain investors, pre-funded warrants to purchase shares of common stock in a private placement offering and an underwritten public offering for aggregate net proceeds of $226.6 million, after placement agent and underwriting fees and offering expenses. These financing events extend our ability to fund our operations and financial obligations into 2028. We expect to continue to incur operating losses as we advance our lead drug candidate through the 4WARD trial. Until we reach profitability, we will need to raise additional capital, which cannot be assured, to fund our operations and meet our financial obligations beyond this period. Such additional capital could be raised through a combination of equity offerings, debt financings, other third-party funding, marketing and distribution arrangements, or 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 and may need to restructure our obligations in a court-supervised process or otherwise.

Due to the numerous risks and uncertainties associated with the future sale of our approved drug product and the research, development, and commercialization of future 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 3 clinical trial of mavorixafor for the treatment of individuals 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 and 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 or 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;
- the costs of acquiring, licensing or investing in businesses, product candidates and technologies;
- the effect of competing technological and market developments; and
- the costs to continue operating as a public company.

Critical Accounting Policies and Significant Judgments and Estimates

Our consolidated financial statements are prepared in accordance with generally accepted accounting principles in the United States. The preparation of our consolidated financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, costs and expenses, and the disclosure of contingent assets and liabilities in our consolidated financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions.

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

Accrued Research and Development Expenses. As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued research and development expenses. This process involves reviewing open contracts and purchase orders, communicating with our applicable personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual costs. The majority of our service providers invoice us in arrears for services performed, on a predetermined 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 us 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 with Arsanis Inc in March 2019 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, and 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 the 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 6 for more information on our goodwill impairment test as of December 31, 2025.

Intangible Assets, Net. Definite-lived intangible assets related to capitalized milestones under license agreements are amortized on a straight-line basis, which aligns with the pattern over which the economic benefit of the intangible assets is consumed, over their remaining useful lives, which are estimated to be the remaining patent life. If our estimate of the product's useful life is shorter than the remaining patent life, then a shorter period is used. Amortization expense is recorded as a component of cost of revenue on the consolidated statements of operations and comprehensive loss.

Revenue Recognition. We recognize revenue when our customers obtain control of the promised good, such as our drug product or licensed intellectual property rights, in an amount that reflects the consideration that we expect to receive in exchange for those goods. We perform the following five steps to determine the amount of revenue to recognize: (1) identify the customer and contract with the customer; (2) identify the performance obligations in the contract; (3) determine the transaction price, adjusted for variable consideration resulting from potential returns, rebates, discounts, and down-stream charges; (4) allocate the transaction price to the performance obligations in the contract; and (5) recognize revenue when we satisfy the performance obligations, which is upon shipment of our drug product to the customer or delivery of licensed rights to the customer.

As part of the accounting for contracts with our customers, we make significant judgments, primarily related to the estimation of the amount of variable consideration to include in the transaction price upon delivery of our drug product or licensed rights. The variable consideration includes estimates for discounts, product returns, and rebates that will be due to U.S. federal and state payors, such as Medicaid, based on agreements that we have with these payors who provide medical insurance to the end patient, and estimated co-pay assistance payments for patients who enroll in our patient assistance program. These variable payments are considered a reduction of the transaction price and must be estimated at the time our product is delivered to the customer. For our license arrangements, we will receive milestone payments based on our achievement of defined operational events or cumulative sales levels achieved. When we conclude that the achievement of such milestones is probable, we include the value of the milestone in the transaction price using the most likely amount method. For product sales, we determine the

amount of variable consideration to include in the transaction price by using the expected value method. Net revenue recognized for each period is the amount for which, based on our estimate, it is probable that a significant reversal of cumulative revenue recognized will not occur. At the end of each subsequent reporting period, we re-evaluate these estimates based on new information and actual operational trends and, if necessary, adjust these variable consideration estimates. Any such adjustments are recorded on a cumulative catch-up basis in the period of the adjustment.

Smaller Reporting Company Status

We are a smaller reporting company ("SRC") as defined by Rule 12b-2 of the Exchange Act and Item 10(f)(1) of Regulation S-K. We may take advantage of certain of the scaled disclosures available to smaller reporting companies 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.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk

As an SRC, we are not required to provide the information requested by 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 that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms and accumulated and communicated to our management, including our principal executive officer and principal financial officer, to allow timely decisions regarding required disclosure.

Our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) as of December 31, 2025, and have concluded that, based on such evaluation, our disclosure controls and procedures were effective as of December 31, 2025 at the reasonable assurance level. Our 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 cost-benefit relationship of possible controls and procedures.

Management's Annual Report on Internal Control over Financial Reporting.

Our management is responsible for establishing and maintaining adequate internal control over financial reporting, as such term is defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act. Our management, including our Chief Executive Officer and Chief Financial Officer, conducted an evaluation of the effectiveness of our internal control over financial reporting based on the criteria set forth in Internal Control-Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission. Based on its evaluation under the criteria set forth in Internal Control-Integrated Framework, our management concluded that our internal control over financial reporting was effective as of December 31, 2025.

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting that occurred during the three months ended December 31, 2025 that have materially affected, or are 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, 2025, no director or Section 16 officer adopted or modified any Rule 10b5-1 trading arrangements or non-Rule 10b5-1 trading arrangements (in each case, as defined in Item 408(a) of Regulation S-K).

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 herein 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," "Executive Officers," "Insider Trading Policy" and, if applicable, "Delinquent Section 16(a) Reports" in our Definitive Proxy Statement on Schedule 14A relating to our 2026 Annual Meeting of Stockholders, which we intend to file with the SEC within 120 days of the end of our fiscal year ended December 31, 2025 (the "2026 Proxy Statement"), and is incorporated herein by reference (excluding pay versus performance disclosure).

Information regarding our Code of Business Conduct and Ethics (the "Code of Conduct") required by this item will be contained in our 2026 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 compliance and ethics section of our website at https://investors.x4pharma.com/corporate-governance/governance-overview. 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 herein by reference to the information set forth in the sections titled "Executive Compensation" in our 2026 Proxy Statement.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters

The information required by this item is incorporated herein 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 2026 Proxy Statement.

Item 13. Certain Relationships and Related Transactions, and Director Independence

The information required by this item is incorporated herein 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 2026 Proxy Statement.

Item 14. Principal Accountant Fees and Services

The information required by this item is incorporated herein by reference to the information set forth in the section titled "Principal Accountant Fees and Services" contained in our 2026 Proxy Statement.

Item 15. Exhibit and Financial Statement Schedules

(1) Financial Statements

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

(2) Financial Statement Schedules:

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

(3) Exhibits

		Incorporated by Reference to:			
Exhibit No.	**Exhibit Description**	**Form**	**Exhibit No.**	**Filing Date**	**File No.**
3.1	Restated Certificate of Incorporation, as amended, as of September 1, 2022.	8-K	3.1	09/01/2022	001-38295
3.2	Certificate of Amendment to the Restated Certificate of Incorporation of X4 Pharmaceuticals, Inc.	8-K	3.1	04/24/2025	001-38295
3.3	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 August 2025 Pre-Funded Warrant.	8-K	4.1	8/12/2025	001-38295
4.3	Form of Pre-Funded Warrant.	8-K	4.1	10/27/2025	001-38295
4.4	Form of Registration Rights Agreement, dated August 11, 2025.	8-K	10.2	8/12/2025	001-38295
4.5	Form of Registration Rights Agreement, dated August 12, 2025.	8-K	10.2	8/13/2025	001-38295
4.6	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
4.7	Description of Registered Securities	10-K	4.17	3/21/2023	001-38295
4.8	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
4.9	Form of July 2022 Pre-Funded Warrant	8-K	4.1	7/1/2022	001-38295
4.10	Form of July 2022 Warrant	8-K	4.2	7/1/2022	001-38295
4.11	Form of December 2022 Pre-Funded Warrant	8-K	4.1	12/9/2022	001-38295

4.12	Form of Class C Warrant.	8-K	4.2	12/9/2022	001-38295
4.13	Form of May 2023 Pre-Funded Warrant.	8-K	4.1	5/16/2023	001-38295
10.1@	Amendment No. 1 to X4 Pharmaceuticals Inc., Amended and Restated 2019 Inducement Equity Incentive Plan.	8-K	10.3	8/12/2025	001-38295
10.2@	Employment Agreement, dated as of August 11, 2025, by and between X4 Pharmaceuticals, Inc. and Adam Craig.	10-Q	10.2	11/5/2025	001-38295
10.3@	Employment Agreement, dated as of August 11, 2025, by and between X4 Pharmaceuticals, Inc. and David Kirske.	10-Q	10.3	11/5/2025	001-38295
10.4@	Employment Agreement, dated as of August 11, 2025, by and between X4 Pharmaceuticals, Inc. and John Volpone.	10-Q	10.4	11/5/2025	001-38295
10.5@	Amended and Restated 2017 Equity Incentive Plan	S-8	99.1	6/10/2020	333-239082
10.6@	Form of Incentive Stock Option Agreement under the 2017 Equity Incentive Plan	S-1	10.8	10/20/2017	001-38295
10.7@	Form of Nonstatutory Stock Option Agreement under the 2017 Equity Incentive Plan	S-1	10.9	10/20/2017	001-38295
10.8@	Form of Restricted Stock Agreement under the 2017 Equity Incentive Plan	8-K	10.6	11/27/2018	001-38295
10.9@	Form of Restricted Stock Unit Agreement under the 2017 Equity Incentive Plan	8-K	10.5	6/19/2019	001-38295
10.10@	Form of Performance-Based Restricted Stock Unit	S-8	99.6	6/10/2020	333-239082
10.11@	X4 Pharmaceuticals Inc. Amended and Restated 2017 Employee Stock Purchase Plan	10-Q	10.4	8/10/2023	001-38295
10.12@	X4 Pharmaceuticals, Inc. 2019 Amended and Restated Inducement Equity Incentive Plan	10-Q	10.3	8/10/2023	001-38295
10.13@	Form of Stock Option Agreement under the 2019 Inducement Equity Incentive Plan	8-K	10.2	6/17/2019	001-38295
10.14@	Form of Restricted Stock Agreement under the 2019 Inducement Equity Incentive Plan	8-K	10.3	6/17/2019	001-38295
10.15@	Form of Restricted Stock Unit Agreement under the 2019 Inducement Equity Incentive Plan	8-K	10.4	6/17/2019	001-38295
10.16@	Form of Indemnification Agreement (for directors and executive officers)	S-1/A	S-1/A	11/06/2017	001-38295
10.17@	Director Compensation Policy	10-K	10.16	3/21/2023	001-38295
10.18@	Amended and Restated Executive Employment Agreement, dated as of March 13, 2019, by and between the Company and Paula Ragan, Ph.D.	8-K	8-K	3/13/2019	001-38295
10.19@	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-Q	3/31/2020	001-38295
10.20@	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
10.21	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

10.22	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
10.23	License Agreement and Supply Agreement, dated as of January 13, 2025, by and between X4 Pharmaceuticals Inc. and Norgine Pharma UK Limited	10-K	10.38#	3/25/2025	001-38295
10.24	Master Services Agreement, dated September 10, 2015, by and between X4 Pharmaceuticals Inc. and Catalent, Inc.	10-K	10.35	3/12/2020	001-38295
10.25	Amendment No. 1 to Master Services Agreement, dated August 25, 2017, by and between X4 Pharmaceuticals Inc. and Catalent, Inc.	10-K	10.36	3/12/2020	001-38295
10.26	Amendment No. 2 to Master Services Agreement, dated February 28, 2020, by and between X4 Pharmaceuticals Inc. and Catalent, Inc.	10-K	10.37	3/12/2020	001-38295
10.27	Amendment No. 3 to Master Services Agreement, dated August 3, 2023, by and between X4 Pharmaceuticals Inc., and Catalent.	10-K	10.37	3/21/2024	001-38295
10.28	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
10.29	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
10.30	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
10.31	Amendment No. 3 to Master Services Agreement, dated February 19, 2024, by and between X4 Pharmaceuticals Inc. and Aptuit (Oxford) Limited.	10-K	10.35	3/25/2025	001-38295
10.32	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
10.33	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
10.34	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
10.35	Amended and Restated Non-Employee Director Compensation Policy, dated February 13, 2024	10-Q	10.16	5/01/2025	001-38295
10.36	Purchase Agreement, dated June 23, 2025, by and between the Company and Lincoln Park Capital Fund, LLC	8-K	10.1	6/23/2025	001-38295
10.37	Registration Rights Agreement, dated June 23, 2025, by and between the Company and Lincoln Park Capital Fund, LLC.	8-K	10.2	6/23/2025	001-38295
19.1	Insider Trading Policy	10-Q	10.3	8/08/2024	001-38295
21.1	List of Subsidiaries	10-K	21.1	3/17/2022	001-38295
23.1*	Consent of PricewaterhouseCoopers LLP, Independent Registered Accounting Firm				
31.1*	Certification of Principal Executive Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.				

31.2*	Certification of Principal Financial Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.				
32.1**	Certification of Principal Executive Officer and Principal Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.				
97.1	Incentive Compensation Recoupment Policy	10-K	97.1	3/21/2024	001-38295
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 and contained in Exhibit 101)				

* Filed herewith
** The certification attached as Exhibit 32.1 accompanying this Annual Report on Form 10-K is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of the Company under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Annual Report on Form 10-K, irrespective of any general incorporation language contained in such filing.
@ Indicates management contract or compensatory plan.

Item 16. Form 10-K Summary

None.

SIGNATURES

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

X4 PHARMACEUTICALS, INC.

Date: March 17, 2026

By: /s/ Adam R. Craig

Adam R. Craig, M.D., Ph.D., M.B.A.
Executive Chairman
(Principal 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/ Adam R. Craig Adam R. Craig, M.D., Ph.D., M.B.A.	President, Chief Executive Officer and Director *(Principal Executive Officer)*	March 17, 2026
/s/ David Kirske David Kirske	Chief Financial Officer and Treasurer *(Principal Financial Officer and Principal Accounting Officer)*	March 17, 2026
/s/ Michael S. Wyzga Michael S. Wyzga	Lead Independent Director	March 17, 2026
/s/ Gary J. Bridger Gary J. Bridger, Ph.D.	Director	March 17, 2026
/s/ Francoise De Craecker Francoise De Craecker	Director	March 17, 2026
/s/ Murray W. Stewart Murray W. Stewart, M.D.	Director	March 17, 2026

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, 2025 and 2024, and the related consolidated statements of operations and comprehensive loss, of stockholders' equity and of cash flows for each of the three years in the period ended December 31, 2025, 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, 2025 and 2024, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2025 in conformity with accounting principles generally accepted in the United States of America.

Basis for Opinion

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

We conducted our audits of these consolidated financial statements in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audits to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. We believe that our audits provide a reasonable basis for our opinion.

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, 2025 was $72.7 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 17, 2026

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, 2025	December 31, 2024
ASSETS		
Current assets:		
Cash and cash equivalents	$ 217,049	$ 55,699
Accounts receivable	573	1,070
Marketable securities	35,949	46,361
Research and development incentive receivable	—	640
Inventory	4,479	2,817
Prepaid expenses and other current assets	3,527	5,588
Total current assets	261,577	112,175
Property and equipment, net	182	776
Goodwill	17,351	17,351
Intangible asset, net	9,250	10,000
Right-of-use assets	1,409	4,065
Other assets	692	2,080
Total assets	$ 290,461	$ 146,447
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 5,696	$ 8,621
Accrued expenses	18,603	23,005
Deferred revenue	464	—
Current portion of lease liability	994	1,251
Total current liabilities	25,757	32,877
Long-term debt, including accretion, net of discount	76,291	75,425
Lease liabilities	—	1,410
Warrant liability	977	13,755
Deferred revenue	621	—
Other liabilities	525	831
Total liabilities	104,171	124,298
Commitments and contingencies (Note 13)		
Stockholders' equity:		
Preferred stock, $0.001 par value. 10,000,000 shares authorized and no shares issued and outstanding as of December 31, 2025 and December 31, 2024, respectively	—	—
Common stock, $0.001 par value, 500,000,000 shares authorized as of December 31, 2025 and December 31, 2024; 90,906,920 and 5,698,231 shares issued and outstanding as of December 31, 2025 and December 31, 2024, respectively	91	6
Additional paid-in capital	780,862	537,620
Accumulated other comprehensive loss	(109)	(122)
Accumulated deficit	(594,554)	(515,355)
Total stockholders' equity	186,290	22,149
Total liabilities and stockholders' equity	$ 290,461	$ 146,447

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,		
	2025	2024	2023
License and other revenue	$ 28,590	$ —	$ —
Product revenue, net	6,523	2,557	—
Total revenue	35,113	2,557	—
Costs and operating expenses:			
Cost of revenue	5,757	797	—
Research and development	72,723	81,643	72,017
General and administrative	43,516	61,518	35,505
Gain on sale of non-financial asset	—	(105,000)	—
Total operating expense	121,996	38,958	107,522
Loss from operations	(86,883)	(36,401)	(107,522)
Other income (expense), net:			
Interest income	4,576	5,769	4,582
Interest expense	(8,876)	(8,768)	(5,777)
Change in fair value of warrant liability	12,778	1,928	7,074
Other (expense) income, net	(753)	332	554
Total other income (expense), net	7,725	(739)	6,433
Loss before provision for income taxes	(79,158)	(37,140)	(101,089)
Provision for income taxes	41	310	78
Net loss	$ (79,199)	$ (37,450)	$ (101,167)
Net loss per share: basic and diluted	$ (1.87)	$ (5.59)	$ (17.07)
Weighted average shares outstanding: basic and diluted	42,292,818	6,702,073	5,927,082
Other comprehensive loss, net of tax:			
Net loss	$ (79,199)	$ (37,450)	$ (101,167)
Change in net unrealized gains on marketable debt securities	13	(3)	—
Comprehensive loss	$ (79,186)	$ (37,453)	$ (101,167)

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

X4 PHARMACEUTICALS, INC.

CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY

(In thousands, except share amounts)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2022	4,055,575	$ 4	$ 450,904	$ (119)	$ (376,738)	**$ 74,051**
Issuance of common stock, warrants and pre-funded warrants for the purchase of common stock, net of issuance costs	1,150,701	1	60,442			**60,443**
Vesting of restricted stock units, less shares withheld and retired to satisfy tax obligations	117,016	—	—			**—**
Exercise of stock options and warrants	249,261	1	8,812			**8,813**
Stock-based compensation expense			8,687			**8,687**
Issuance of shares of common stock under employee stock purchase plan	8,600		272			**272**
Net loss					(101,167)	**(101,167)**
Balance at December 31, 2023	5,581,153	$ 6	$ 529,117	$ (119)	$ (477,905)	**$ 51,099**
Issuance of common stock under employee stock purchase plan	21,185	—	297			**297**
Vesting of restricted stock units	95,893	—	2			**2**
Stock-based compensation			8,204			**8,204**
Unrealized loss on marketable securities				(3)		**(3)**
Net loss					(37,450)	**(37,450)**
Balance at December 31, 2024	5,698,231	$ 6	$ 537,620	$ (122)	$ (515,355)	**$ 22,149**
Issuance of common stock and pre-funded warrants for the purchase of common stock, net of issuance costs	69,688,750	70	238,855			**238,925**
Exercise of pre-funded warrants	15,241,246	15	—			**15**
Issuance of common stock under employee stock purchase plan	38,769	—	89			**89**
Vesting of restricted stock units	239,924	—	—			**—**
Stock-based compensation			4,298			**4,298**
Unrealized losses on marketable securities				13		**13**
Net loss					(79,199)	**(79,199)**
Balance at December 31, 2025	**90,906,920**	**$ 91**	**$ 780,862**	**$ (109)**	**$ (594,554)**	**$ 186,290**

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,		
	2025	**2024**	**2023**
Cash flows from operating activities:			
Net loss	$ (79,199)	$ (37,450)	$ (101,167)
Adjustments to reconcile net loss to net cash used in operating activities:			
Stock-based compensation expense	4,298	8,204	8,687
Depreciation and amortization expense	1,277	796	419
Gain on sale of non-financial asset	—	(105,000)	—
Non-cash lease expense	2,656	1,585	1,577
Accretion of debt discount	865	856	929
Change in fair value of warrant liability	(12,778)	(1,928)	(7,074)
Other	(161)	(639)	(227)
Changes in operating assets and liabilities:			
Accounts receivable	497	(1,070)	—
Inventory	(1,662)	(2,817)	—
Prepaid expenses, other current and non-current assets and research and development incentive receivable	4,248	683	(1,370)
Accounts payable	(2,979)	(286)	1,234
Accrued expenses and other long-term liabilities	(2,025)	7,169	1,608
Deferred revenue	1,085	—	—
Lease liabilities	(1,740)	(1,004)	(1,128)
Net cash used in operating activities	(85,618)	(130,901)	(96,512)
Cash flows from investing activities:			
Acquisition of intangible asset	(3,000)	(7,000)	—
Proceeds from sale of non-financial asset	—	105,000	—
Purchase of marketable securities	(63,388)	(57,134)	(16,823)
Sales and maturities of marketable securities	74,542	26,450	2,000
Acquisition of property and equipment	—	(326)	(60)
Net cash provided by (used in) investing activities	8,154	66,990	(14,883)
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 plan	98	294	8,712
Proceeds from borrowings under loan and security agreement	—	20,000	22,500
Repayments of borrowings under loan and security agreement	—	—	(2,064)
Issuance costs for amendments to loan and security agreement and for the sale of warrants accounted as a liability	—	—	(631)
Proceeds from sale of shares of common stock, warrants and pre-funded warrants, less issuance costs	238,530	—	59,999
Net cash provided by financing activities	238,628	20,294	88,516
Effect of exchange rate changes on cash, cash equivalents and restricted cash	213	(156)	99
Net increase (decrease) in cash, cash equivalents and restricted cash	161,377	(43,773)	(22,780)
Cash, cash equivalents and restricted cash at beginning of period	56,475	100,248	123,028
Cash, cash equivalents and restricted cash at end of period	$ 217,852	$ 56,475	$ 100,248
Acquisition of intangible assets included in accrued expenses	$ 500	$ 3,500	$ —
Cash paid for interest	$ 8,053	$ 7,766	$ 4,604

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

1. Description of Business and Basis of Presentation

X4 Pharmaceuticals Inc., together with its subsidiaries, also referred to in these financial statements as "we," "us," "our," the "Company" and "X4," is a biopharmaceutical company developing and commercializing novel therapeutics for the treatment of rare hematology diseases. The Company continues to progress its global, pivotal Phase 3 clinical trial, (the "4WARD" trial) to evaluate the efficacy, safety, and tolerability of oral, once-daily mavorixafor (with or without stable doses of granulocyte colony-stimulating factor ("G-CSF")) in people with congenital, acquired primary autoimmune, or idiopathic chronic neutropenia ("CN") who are experiencing recurrent and/or serious infections. The 52-week trial is a randomized, double-blind, placebo-controlled, multicenter study aiming to enroll up to 176 patients, with full enrollment expected in the third quarter of 2026. The U.S. Food and Drug Administration ("FDA") has granted Fast Track designation to mavorixafor for the treatment of CN, which 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). CN 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 Company has one commercially approved product, XOLREMDI ® (mavorixafor), which has received accelerated approval in the United States from the FDA for use as an oral, once-daily therapy in patients 12 years of age and older with WHIM (warts, hypogammaglobulinemia, infections, and myelokathexis) syndrome, to increase the number of circulating mature neutrophils and lymphocytes. WHIM syndrome is a rare combined primary immunodeficiency and chronic neutropenic disorder. The Company is committed to making XOLREMDI available to patients in need in the U.S. while maintaining its focus on its long-term strategy to successfully complete the 4WARD trial in patients with moderate and severe CN. The Company is headquartered in Boston, Massachusetts.

Basis of Presentation

On April 28, 2025, the Company effected a 1-for-30 reverse stock split of its common stock (the "Reverse Stock Split"). Unless otherwise noted, all references to common stock share and per share amounts in this Annual Report on Form 10-K have been retroactively adjusted to reflect the Reverse Stock Split.

Principles of Consolidation

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

Liquidity

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. Although the Company has an approved drug product, licensing and sales of its drug product over the next 12 months will not be sufficient to fund its operating expenses. Since inception, the Company has incurred significant operating losses and negative cash flows from operations, and the Company expects to continue to generate operating losses and negative cash flows from operations for the foreseeable future. For the year ended December 31, 2025, the Company's net loss was $79.2 million and net cash used in operating activities was $85.6 million. As of December 31, 2025, the Company had $253.0 million of cash, cash equivalents and short-term marketable securities, and an accumulated deficit of $594.6 million.

Based on its current operating plan, the Company believes (a) that its current cash, cash equivalents and short-term marketable will be sufficient to fund its operations for at least the next 12 months and (b) it will continue to comply with all covenants under the Hercules Loan Agreement, as defined and further described in Note 11, through at least the 12-month period from the issuance date of these consolidated financial statements.

The Company is subject to risks common to companies in the biopharmaceutical industry including, but not limited to, uncertainties relating to conducting preclinical and clinical research and development, the manufacture and supply of products and product candidates for clinical and commercial use, obtaining and maintaining regulatory approvals and pricing and reimbursement for its products and product candidates, market acceptance, managing global growth and operating expenses, availability of additional capital, competition, obtaining and enforcing patents, stock price volatility, dependence on

collaborative relationships and third-party service providers, dependence on key personnel, and from time to time government investigations, litigation, and potential product liability claims.

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 and reporting currency of the Company and its foreign subsidiary, X4 Austria, is the U.S. dollar. Monetary assets and liabilities denominated in a currency other than the U.S. dollar are re-measured into U.S. dollars at the exchange rate prevailing as of the balance sheet date. Non-monetary assets and liabilities acquired in a currency other than U.S. dollars are measured at historical exchange rates prevailing at each transaction date. Exchange gains and losses on translation are included in the consolidated statements of operations and comprehensive loss in other income (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 and marketable securities. 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 and commercial drug supply for research and development activities in its programs and for commercial sale. The Company relies and expects to continue to rely on a small number of manufacturers to supply it the active pharmaceutical ingredient and formulated drugs related to its commercial drug supply and for its development these programs. The Company's sales and 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, 2025 and December 31, 2024.

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 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 the Company's 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, 2025	As of December 31, 2024
Letter of credit security: Vienna Austria lease	$ 224	$ 199
Letter of credit security: Boston lease	579	577
Total restricted cash	$ 803	$ 776

In connection with the Company's lease agreements for its facilities in Boston, Massachusetts and Vienna, Austria, the Company maintains letters of credit, which are secured by restricted cash, for the benefit of the respective landlord. As of December 31, 2025, restricted cash related to the Vienna, Austria lease is included in other current assets and was subsequently converted to cash prior to the issuance of these consolidated financial statements. Restricted cash related to the Boston lease is also included in other current assets.

In accordance with the Company's Hercules Loan Agreement and as further described in Note 11, the Company at all times must maintain a minimum level of cash of $15.0 million in an account or accounts in which Hercules Capital Inc. ("Hercules") has a first priority security interest.

The following table provides a reconciliation of cash, cash equivalents, and restricted cash reported within 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, 2025 and December 31, 2024:

(in thousands)	December 31, 2025	December 31, 2024
Cash and cash equivalents	$ 217,049	$ 55,699
Restricted cash, current (included within prepaid expenses and other current assets)	803	—
Restricted cash (non-current)	—	776
Total cash, cash equivalents and restricted cash	$ 217,852	$ 56,475

Accounts Receivable— Accounts receivable consists of amounts due from customers, net of expected credit losses. The Company's measurement of expected credit losses is based on relevant information about past events, including historical experience, current conditions, and reasonable and supportable forecasts that affect the collectability of the reported amount. To date, the Company has not experienced any credit losses from its customers. The Company's contracts with its customers have customary payment terms that generally require payment within 90 days. The Company analyzes amounts that are past due for collectability, and periodically evaluates the creditworthiness of its customers. At December 31, 2025 and December 31, 2024, the Company determined an allowance for credit losses was not required based on the deemed credit worthiness of its customers.

Inventory— Prior to receiving approval from the FDA in April 2024 to sell XOLREMDI (mavorixafor) in the United States, the Company expensed all costs incurred related to the manufacture of mavorixafor as research and development expense due to the inherent risks associated with the development of a drug candidate, the uncertainty about the regulatory approval process and the lack of history for the Company in obtaining of regulatory approval of drug candidates. The Company has capitalized inventory-related costs that have been incurred subsequent to FDA approval, such as the bottling, labelling, and packaging of drug product, and the acquisition of raw materials for the production of drug substance to be used in commercial drug product.

In connection therewith, the Company values inventories at the lower of cost or estimated net realizable value. The Company determines the cost of inventories on a first-in, first-out ("FIFO") basis. Raw materials and work in process include all inventory costs prior to packaging, and labelling, including raw materials and the active pharmaceutical ingredient used in the drug product. Finished goods include packaged and labelled drug products designated for commercial distribution. Clinical drug supplies are expensed to research and development. Raw materials and work in process that may be used for either research and development or commercial sale are classified as inventory until the material is consumed or otherwise allocated for research and development. If the material may only be used for research and development, it is expensed as research and development.

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

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. Pursuant to a policy election, 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, operating lease right-of-use assets and definite-lived intangible 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 6 for more information on the Company's goodwill impairment tests as of December 31, 2025, 2024 and 2023.

Intangible Assets, Net— Definite-lived intangible assets related to capitalized milestones under license agreements are amortized on a straight-line basis, which aligns with the pattern over which the economic benefit of the intangible assets are consumed, over their remaining useful lives, which are estimated to be the remaining patent life. If the Company's estimate of the product's useful life is shorter than the remaining patent life, then a shorter period is used. Amortization expense is recorded as a component of cost of revenue on the consolidated statements of operations and comprehensive loss.

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, 2025 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 Executive Chairman. 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. See Note 20 for the Company's segment disclosures required by ASC 280, *Segment Reporting*.

Revenue Recognition— The Company records revenue using the guidance of ASC Topic 606, *Revenue from Contracts with Customers* ("ASC 606"), as amended. Upon the approval by the FDA of the sales and marketing of the Company's lead product candidate, revenue related to its sale and distribution is accounted for under ASC 606. The Company recognizes revenue when its customer obtains control of promised goods, services or licensed rights, in an amount that reflects the consideration that the Company determines it expects to receive in exchange for those goods, services or licensed rights. To determine revenue recognition for arrangements that the Company determines are within the scope of ASC 606, the Company performs the following five steps: (1) identify the customer and contract with the customer; (2) identify the performance obligations in the contract; (3) determine the transaction price, adjusted for variable consideration resulting from potential returns, rebates, discounts, down-stream charges and the probability of achievement of future milestones; (4) allocate the transaction price to the performance obligations in the contract; and (5) recognize revenue when the Company satisfies its performance obligations, which is upon delivery of the finished product to the customer, delivery of services or delivery of licensed rights.

The Company currently sells its product to a specialty pharmacy, which dispenses the Company's drug product to patients in the U.S. The Company records revenue when the specialty pharmacy obtains control over the promised good, which occurs at a point in time, typically upon delivery to the specialty pharmacy. The Company has concluded that it provides one performance obligation in its contract with the specialty pharmacy customer and for other customers in this class: the delivery of drug product that has been approved for sale and distribution by the applicable regulatory authority.

The Company has also entered into a license and supply agreement (the "Norgine Agreement") with Norgine Pharma UK Ltd. ("Norgine"). The terms of the Norgine Agreement contain multiple performance obligations, which include (a) the delivery of a license, (b) WHIM research and development services and (c) CN research and development services. Payments to the Company under this arrangement include non-refundable, upfront license fees; regulatory and sales-based milestone payments and royalties on future product sales.

As part of the accounting for its contract arrangements, the Company makes significant judgments, primarily related to the estimation of the amount of variable consideration to include in the transaction price upon delivery of the Company's drug product or licensed rights.

- The variable consideration for product sales typically includes estimates for discounts, product returns, rebates due to U.S. federal and state payors, such as Medicaid, based on agreements that the Company has with these payors who provide medical insurance to the end patient, and estimated co-pay assistance payments for patients who enroll in the Company's patient assistance program. These variable payments are considered a reduction of the Company's transaction price with its customer and are estimated using the expected value method at the time the Company's product is delivered to the customer.
- The consideration in license agreements typically includes future regulatory milestone payments, sales-based milestone payments, and royalties on future product sales. The Company has elected to exclude sales-based milestone payments and sales-based royalties from the transaction price under the applicable practical expedient. As such, the remaining variable consideration is generally comprised of future regulatory or operational milestone payments, which are considered for inclusion in the transaction price at the outset of the arrangement and at each reporting period using the most-likely-amount method.

For arrangements which contain multiple performance obligations, the Company allocates the transaction price to each performance obligation based on the estimated relative standalone selling price. The Company estimates the standalone selling price with the objective of determining the price at which the Company would sell such an item if it were to be sold regularly on a standalone basis.

Net revenue recognized for each period is the amount for which, based on management's estimate, it is probable that a significant reversal of cumulative revenue recognized will not occur. At the end of each subsequent reporting period, the Company re-evaluates these estimates based on new information and actual operational trends and if necessary, adjusts these variable consideration estimates. Any such adjustments are recorded on a cumulative catch-up basis in the period of the adjustment.

Cost of Revenue— Cost of revenue consists of drug product costs, including capitalized internal direct and overhead costs associated with the manufacturing, lot release and distribution of XOLREMDI, amortization of intangible assets associated with license agreements and royalty expense. Cost of revenue may also include costs related to excess or obsolete inventory adjustment charges and abnormal manufacturing costs.

Costs to Obtain a Contract with a Customer— The Company capitalizes incremental costs to obtain a contract with a customer, such as external finder's fees and commissions. Such costs are amortized to sales and marketing expense within general and administrative costs over the estimated life of the customer relationship in proportion to the value of license, products and services provided.

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. This process involves reviewing open contracts and purchase orders, communicating with the Company's applicable personnel to identify services that have been performed on its behalf 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.

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, which model includes (a) stock price volatility, based on the historical volatility of the Company's stock price for the duration of the expected life of the option, (b) the expected term, which is based "simplified" method for awards that qualify as "plain-vanilla" options, (c) the risk-free interest rate, which 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 and (d) expected dividend yield, which 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 Hercules Loan Agreement 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 (expense) income, net, in the consolidated statements 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 years ended December 31, 2025, 2024, and 2023, 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. As of December 31, 2025, unrealized gains on the Company's marketable debt security portfolio are included in accumulated other comprehensive loss on the consolidated balance sheet. Once these gains (losses) are realized, they are included in other (expense) income, net, on the consolidated statements 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. 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
In December 2023, the Financial Accounting Standards Board ("FASB") issued ASU 2023-09, *Income Taxes (Topic 740): Improvements to Income Tax Disclosures (*"ASU 2023-09"). ASU 2023-09 requires an entity's annual financial statements to include consistent categories and greater disaggregation of information in the rate reconciliation, and income taxes paid disaggregated by jurisdiction. ASU 2023-09 is effective for fiscal years beginning after December 15, 2024, with early adoption permitted. Adoption is either with a prospective method or a fully retrospective method of transition. The Company has adopted ASU 2023-09 for the year ended December 31, 2025, and applied the new disclosure requirements prospectively to the current annual period. Prior period disclosures have not been adjusted to reflect the new disclosure requirements. Please see additional disclosures related to income taxes in Note 17, *Income Taxes.*

Recently Issued Accounting Standards Not Yet Adopted
In November 2024, the FASB issued ASU 2024-03, *Disaggregation of Income Statement Expenses (Topic 220)* ("ASU 2024-03") requiring that public business entities disclose additional information about specific expense categories in the notes to financial statements at interim and annual reporting periods. The amendments in ASU 2024-03 are effective for annual reporting periods beginning after December 15, 2026, and interim reporting periods beginning after December 15, 2027. The requirements in ASU 2024-03 may be applied either prospectively to financial statements issued for reporting periods after the effective date or retrospectively to any or all prior periods presented in the financial statements. The Company continues to evaluate the impact of ASU 2024-03.

3. Strategic Restructurings

In the first quarter of 2025, the Company implemented a strategic restructuring of its business operations, workforce and capital spending to focus efforts on advancing mavorixafor to treat patients with CN. As part of this restructuring, the Company (i) implemented a net reduction of its employee headcount by 43 employees, or approximately 30% of its total workforce as of February 2025, including its U.S. commercial field team, (ii) commenced the closure of its research and development facility in Vienna, Austria, (iii) paused its pre-clinical drug candidate programs and (iv) streamlined other spending to support the ongoing clinical development of mavorixafor for the larger population of patients with CN. In addition, in the third quarter of 2025, the Company implemented additional strategic restructuring designed to sharpen its operational focus and align resources with the Company's long-term strategy to successfully complete the 4WARD trial in patients with moderate and severe CN. As part of this initiative, the Company further reduced its workforce by an additional approximately 50%. This workforce reduction was substantially completed in the third quarter of 2025.

The Company incurred charges of $7.0 million for severance and other employee termination-related costs related to these strategic restructuring actions, of which $2.0 million is included in research and development and $5.0 million is included in general and administrative expense on the accompanying consolidated statement of operations for the year ended December 31, 2025. During the year ended December 31, 2024, as a result of operational reorganizations, the Company incurred severance and other benefit charges due to employee terminations in the amount of $0.9 million.

Approximately $1.5 million of accrued severance and other employee termination-related costs are included in accrued expenses on the consolidated balance sheet as of December 31, 2025.

The following table summarizes the Company's liability recognized in connection with these headcount reductions:

(in thousands)		
Balance as of December 31, 2023	$	94
Severance and other employee-related expenses		861
Cash payments		(485)
Balance as of December 31, 2024		470
Severance and other employee-related expenses		7,007
Cash payments		(5,951)
Balance as of December 31, 2025	$	1,526

4. License Agreements and Funding Arrangements

Research and Development Incentive Program

The Company participated in a research and development incentive program provided by the Austrian government whereby the Company was entitled to reimbursement by the Austrian government for a percentage of qualifying research and development expenses and capital expenditures incurred by the Company's subsidiary in Austria. As of December 31, 2025, no amounts were due under the program. As of December 31, 2024, $0.6 million was due under the program, which is included in research and development incentive receivable on the consolidated balance sheet. During the year ended December 31, 2025, no amounts were recorded for this program as the Company is in the process of closing the Vienna site. During the years ended December 31, 2024 and 2023, the Company recorded $0.7 million and $0.6 million, respectively, of income related to the program within the consolidated statement of operations as other income.

Genzyme Agreement

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

Under the Genzyme Agreement, the Company is obligated to make milestone payments contingent upon the achievement of certain regulatory and sales milestones with respect to licensed products. During the year ended December 31, 2025, a $3.0 million regulatory milestone, which had been previously accrued, was paid after receipt of the European Medicines Agency's ("EMA") validation of the Company's Marketing Authorisation Application ("MAA") for processing. The payment was recorded as a definite-lived intangible asset as discussed in Note 9.

As of December 31, 2025, the Company is obligated to make future milestone payments in the aggregate amount of up to $10.0 million, including $5.0 million upon the notification by the EMA of regulatory approval of the Company's MAA for mavorixafor for the WHIM indication, and 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. As of December 31, 2025, the Company accrued $0.5 million of sales-based milestones as a component of the definite-lived intangible asset, as management has concluded that the achievement of this milestones is probable.

In addition, for the year ended December 31, 2025, the Company incurred $4.5 million of fees under the Genzyme Agreement as a result of sublicense payments received and sales of licensed product. Such fees were recorded as cost of revenue.

The Company is 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 incurs royalties on annual net sales at a rate of 6% up to $150.0 million, 10% on the portion of annual net sales between $150.0

million and $300.0 million, and 12% thereafter on annual sale over $300.0 million. The Company also incurs a 15% royalty on certain sublicense payments received from sub-licensees.

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.

Norgine Agreement

On January 13, 2025, the Company entered into the Norgine Agreement, pursuant to which Norgine was granted an exclusive license to seek regulatory approval and distribute, market and sell the Company's product mavorixafor within the Field (as defined in the Norgine Agreement), in the European Economic Area, Switzerland, the United Kingdom, Australia and New Zealand (collectively, the "Territory"), following regulatory approval in the Territory. Additionally, Norgine was granted a co-exclusive license to manufacture mavorixafor for the Territory. The Company retains all rights to mavorixafor outside the Territory and specific reserved rights within the Territory. Norgine may grant sublicenses to its affiliates and certain third parties subject to the terms of the Norgine Agreement, except that it may not sublicense the commercial rights granted under the Norgine Agreement for certain countries without X4's explicit consent.

Pursuant to the terms of the Norgine Agreement, the Company received a one-time, non-refundable, upfront payment of €28.5 million and a regulatory milestone payment of €0.5 million. The Company could receive up to approximately €20.6 million, €20.0 million and €185.0 million upon the achievement of certain regulatory, commercial, and sales milestones, respectively, or €225.6 million in aggregate. The Norgine Agreement also includes escalating double-digit royalties of up to mid-twenties on any future net sales in the Territory. The tiered royalty payments are subject to royalty stacking, and to a material reduction on a country-by-country basis if a generic version of mavorixafor becomes available in the applicable country. The Company and Norgine will collaborate closely on regulatory filings, with the Company continuing to be responsible for the ongoing 4WARD clinical trial evaluating mavorixafor in CN and certain components of pediatric studies for WHIM. Norgine will be responsible for all market access and commercialization activities. The Company also agreed to manufacture and supply mavorixafor to Norgine. Norgine is required to pay a supply price to the Company for the licensed product derived from the Company's manufacturing costs plus margin in the low teens.

Subject to customary rights of each party to earlier terminate the Norgine Agreement, the term of the Norgine Agreement continues, on a country-by-country basis, until the later of: (i) the tenth (10th) anniversary of the first commercial sale of mavorixafor, (ii) expiration of regulatory market exclusivity of mavorixafor or (iii) expiration of the last-to-expire licensed patent in such country. The term of the Norgine Agreement shall be automatically renewed for additional three-year terms unless either party provides the other party written notice of its intent not to renew the Norgine Agreement at least one year prior to the applicable termination date of the Norgine Agreement. In the event of automatic renewal, the royalty payment rate drops to a single digit royalty.

5. Revenue

Product Revenue, Net

During the years ended December 31, 2025 and 2024, the Company recorded net revenue $6.5 million and $2.6 million, respectively, for the sale of its drug product in the U.S. There were no sales of drug product for the year ended December 31, 2023.

The following table summarizes the balances and activity in each of the product reserve accounts for the years ended December 31, 2025 and 2024.

(in thousands)	Rebates and Discounts	Co-Pay Assistance	Product Returns	Total
Balance at December 31, 2023	$ —	$ —	$ —	$ —
Provision related to revenue associated with sales processed during the year ended December 31, 2024	185	63	11	259
Credits and payments made during the period	(86)	(34)	—	(120)
Balance at December 31, 2024	99	29	11	139
Provision related to revenue associated with sales processed during the year ended December 31, 2025	653	100	40	793
Adjustments related to revenue associated with sales recognized during prior periods	(16)	10	(41)	(47)
Credits and payments made during the period	(318)	(109)	—	(427)
Balance as of December 31, 2025	$ 418	$ 30	$ 10	$ 458

The provision for contractual discounts provided to the Company's customer is recorded as a reduction of accounts receivable. The provisions for co-pay assistance payments, contractual rebates and product returns are classified within accrued expenses.

The following table provides a rollforward of accounts receivable for the years ended December 31, 2025 and 2024.

(in thousands)	Accounts Receivable
Beginning balance at December 31, 2023	$ —
Increase in accounts receivable for drug product sales	2,735
Decrease in accounts receivable for cash collections	(1,665)
Beginning balance at December 31, 2024	1,070
Increase in accounts receivable for drug product sales	7,062
Decrease in accounts receivable for cash collections	(7,559)
Balance as of December 31, 2025	$ 573

License and Other

During the first quarter of 2025, the Company entered into the Norgine Agreement. The Company analyzed the activities required under the Norgine Agreement and concluded that the arrangement was indicative of a vendor-customer relationship and would be accounted for under ASC 606. During the year ended December 31, 2025, the Company received a one-time, non-refundable, up-front payment of €28.5 million and a regulatory milestone payment of €0.5 million, which are included the transaction price. All other future regulatory-based milestone payments, which represent variable consideration, have been fully constrained as these are not yet considered probable. The Company has also excluded from the transaction price future royalty payments that are based on units sold by Norgine and future cumulative revenue-based milestone payments under the applicable practical expedient.

Under the Norgine Agreement, the Company's promises include a) the delivery of a license, b) research and development services for certain components of WHIM clinical studies, c) research and development services for the global Phase 3 trial of mavorixafor for CN, and d) the option for delivery of commercial drug supply pursuant to a manufacturing agreement. The Company assessed the above promises and determined that the option for delivery of commercial drug supply is priced at fair value, and therefore is not a material right or a performance obligation. Revenue from the delivery of manufacturing supply will

be recorded when delivered at the pricing agreed to in the contract. The license was considered functional intellectual property as of the inception of the Norgine Agreement and distinct from other promises under the contract, as Norgine can benefit from the license on its own or together with other readily available resources. Each of the research and development services were considered distinct as the customer can benefit from these services together with the license transferred at the inception of the agreement. The research and development services will not modify or customize the initial intellectual property transferred at contract inception due to the late stage of development of the intellectual property. As a result, the Company identified three performance obligations: (a) the delivery of the license, (b) research and development services for certain components of WHIM clinical studies, and (c) research and development services for the global Phase 3 trial of mavorixafor for CN.

The Company allocated the transaction price of $29.7 million among these three performance obligations based on the Company's best estimate of stand-alone selling price for each distinct performance obligation. The Company developed the estimated standalone selling price, at inception, for each of the three performance obligations with the objective of determining the price at which the Company would sell such an item if it were to be sold regularly on a standalone basis. The Company developed the estimated standalone selling price for the license primarily based on the probability-weighted present value of expected future cash flows. In developing such estimates, the Company applied judgment in determining the forecasted revenues, taking into consideration the applicable market conditions and relevant entity-specific factors, the probability of success, the time needed to develop mavorixafor and the discount rate. The Company developed the estimated standalone selling price for the research and development services based on the amount a third party would pay for these services, which contemplates the level of efforts necessary to perform these services and the costs for full-time equivalent employees and expected resources to be committed plus a reasonable margin.

During the year ended December 31, 2025, the Company recognized $27.6 million for the delivery of the license and $1.0 million for research and development services. The license performance obligation was satisfied at a point in time upon transfer of the license to Norgine. Control of the license was transferred on the effective date of the Norgine Agreement as Norgine could begin to use and benefit from the license. For the research and development performance obligations, the Company recognizes revenue over time using an input method based on cost incurred during the period relative to the total estimated cost of the obligation. This method, in management's judgment, is the best measure of progress towards satisfying the performance obligation as the transfer of control occurs as services are performed. The amounts received that have not yet been recognized as revenue are recorded as deferred revenue on the consolidated balance sheet and will be recognized over the remaining period as the performance obligation is satisfied.

The following table summarizes the allocation of transaction price to the three performance obligations in the Norgine Agreement based on the weighting of estimated stand-alone selling price for these performance obligations at the inception of the agreement.

(in thousands)		Revenue Recognized Year Ended December 31,		
Performance Obligation:	**Allocation of Transaction Price**	**2025**	**2024**	**2023**
License	$ 27,639	$ 27,639	$ —	$ —
Research and development services: WHIM	312	25	—	—
Research and development services: CN	1,724	926	—	—
Total	$ 29,675	$ 28,590	$ —	$ —

As of December 31, 2025, deferred revenue related to the Norgine Agreement was $1.1 million, of which $0.5 million was current.

During the year ended December 31, 2025, the Company capitalized $4.0 million of incremental costs to obtain a contract with a customer and amortized $3.3 million of these costs through general and administrative expense for the year ended December 31, 2025. No such costs were capitalized or amortized during the years ended December 31, 2024 and 2023. As of December 31, 2025, unamortized capitalized costs to obtain a contract with a customer on the consolidated balance sheet were $0.7 million, of which $0.2 million were classified in other current assets and $0.5 million were classified in other assets.

6. 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, 2025 Using:			
(in thousands)	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents—money market funds	$ 123,838	$ —	$ —	$ 123,838
Marketable securities— U.S. Treasury notes, U.S. Treasury bills, and federal government agency notes	—	35,949	—	35,949
	$ 123,838	$ 35,949	$ —	$ 159,787
Liabilities:				
Embedded derivative liability	$ —	$ —	$ 10	$ 10
Class C warrant liability	—	—	977	977
	$ —	$ —	$ 987	$ 987

	Fair Value Measurements as of December 31, 2024 Using:			
(in thousands)	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents—money market funds	$ 40,983	$ —	$ —	$ 40,983
Marketable securities—U.S. Treasury notes, U.S. Treasury bills, and federal government agency notes	—	46,361	—	46,361
	$ 40,983	$ 46,361	$ —	$ 87,344
Liabilities:				
Embedded derivative liability	$ —	$ —	$ 10	$ 10
Class C warrant liability	—	—	13,755	13,755
	$ —	$ —	$ 13,765	$ 13,765

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, approximated 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, 2025 and December 31, 2024.

The Company's cash equivalents consisted of money market funds invested 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.

The following table provides amortized cost, unrealized gains and losses and the carrying amount of available-for-sale debt marketable securities as of December 31, 2025:

(in thousands)	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
U.S. Treasury securities	$ 7,959	$ 3	$ —	$ 7,962
Federal Government Agency securities	27,980	11	4	27,987
Total available-for-sale debt securities	$ 35,939	$ 14	$ 4	$ 35,949

The following table provides amortized cost, unrealized gains and losses and the carrying amount of available-for-sale debt marketable securities as of December 31, 2024:

(in thousands)	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
U.S. Treasury securities	$ 18,928	$ 5	$ 3	$ 18,930
Federal Government Agency securities	27,436	9	14	27,431
Total available-for-sale debt securities	$ 46,364	$ 14	$ 17	$ 46,361

The following table provides a roll-forward for the three years ended December 31, 2025, of the aggregate fair values financial instruments for which fair values are determined using Level 3 inputs:

(in thousands)	Embedded Derivative Liability	Class C Warrant Liability	Total
Balance as of December 31, 2022	$ 10	$ 23,131	$ 23,141
Reclassification to permanent equity upon exercise	—	(374)	(374)
Change in fair value	—	(7,074)	(7,074)
Balance as of December 31, 2023	10	15,683	15,693
Change in fair value	—	(1,928)	(1,928)
Balance as of December 31, 2024	10	13,755	13,765
Change in fair value	—	(12,778)	(12,778)
Balance as of December 31, 2025	$ 10	$ 977	$ 987

Valuation of Embedded Derivative Liability— The fair value of the embedded derivative liability recognized in connection with the Company's Hercules Loan Agreement, which is associated with additional fees due to Hercules upon non-credit related 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%.

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 "change in fair value of warrant liability" on the consolidated statements of operations and comprehensive loss.

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

	Class C Warrants		
	December 31, 2025	December 31, 2024	December 31, 2023
Common stock price	$4.00	$21.90	$25.20
Risk-free interest rate	3.5 %	4.2 %	3.9 %
Expected term (in years)	1.9	2.9	3.9
Expected volatility	136.0 %	117.5 %	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. The Company tested goodwill for

impairment as of December 31, 2025, 2024 and 2023 and concluded that goodwill was not further impaired. Should the market value of the Company's common stock decline, 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, 2023	$ 27,109	$ (9,758)	$ 17,351
Goodwill at December 31, 2024	27,109	(9,758)	17,351
Goodwill at December 31, 2025	$ 27,109	$ (9,758)	$ 17,351

7. Inventory

Inventory consists of the following:

(in thousands)	December 31, 2025	December 31, 2024
Raw materials	$ 1,382	1,529
Work in process	2,518	608
Finished goods	579	680
Total inventory	$ 4,479	$ 2,817

8. Property and Equipment

Property and equipment, net consisted of the following:

(in thousands)	December 31, 2025	December 31, 2024
Leasehold improvements	$ 228	$ 228
Furniture and fixtures	1,035	1,472
Computer equipment	134	286
Software	—	24
Lab equipment	17	669
	1,414	2,679
Less: Accumulated depreciation	(1,232)	(1,903)
	$ 182	$ 776

Depreciation expense related to property and equipment was $0.5 million, $0.3 million and $0.4 million for the years ended December 31, 2025, 2024 and 2023, respectively.

9. Intangible Assets, Net

As of December 31, 2025, the Company's net definite-lived intangible asset, which includes the capitalization of certain milestone payments made or accrued related to its license agreement for the intellectual property contained in its drug product, includes a gross intangible asset of $10.5 million, less accumulated amortization of $1.3 million for a net intangible asset of $9.3 million. The Company amortizes the intangible asset to cost of revenue over the remaining life of the underlying patent protecting the intellectual property through 2038.

As of December 31, 2025, amortization expense for the next five years and beyond is summarized as follows:

(in thousands) Year	Amortization
2026	$ 750
2027	750

2028	750
2029	750
2030	750
Thereafter	5,500
Total	$ 9,250

The Company began amortizing its finite-lived intangible assets in April 2024 over a 14-year period based on the expected patent exclusivity period for XOLREMDI. Amortization expense totaled $0.8 million and $0.5 million for the years ended December 31, 2025 and 2024, respectively. Amortization expense is recorded as a component of cost of revenue on the consolidated statements of operations and comprehensive loss.

10. Accrued Expenses

Accrued expenses consisted of the following:

(in thousands)	December 31, 2025	December 31, 2024
Accrued employee compensation and benefits	$ 4,303	13,053
Accrued external research and development expenses	12,235	3,727
Accrued royalty and milestone payments	144	3,092
Accrued professional fees	816	1,825
Other	1,105	1,308
	$ 18,603	$ 23,005

11. Long-Term Debt

Long-term debt consisted of the following:

(in thousands)	December 31, 2025	December 31, 2024
Principal amount of long-term debt	$ 75,000	$ 75,000
Debt discount, net of accretion	(412)	(650)
Cumulative accrual of end of term payments	1,703	1,075
Long-term debt	$ 76,291	$ 75,425

Hercules Loan Agreement

The Company is party to the Second Amended and Restated Loan and Security Agreement, as amended, (the "Hercules Loan Agreement") with Hercules, which provides for an aggregate term loan facility of up to $107.5 million, under which the Company has borrowed an aggregate of $75.0 million of term loans, representing the maximum borrowings allowable as of December 31, 2025. Additional borrowings are available subject to approval of the Lender (as defined in the Hercules Loan Agreement) 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 July 1, 2027, which is the maturity date of the Hercules Loan Agreement. 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 1%. In addition, the Hercules Loan Agreement provides for payment of end-of-term fees of $2.8 million plus 3.5% of the aggregate principal amount of loans drawn, if any, subsequent to the most recent amendment in August 2023, 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. The Company must maintain cash, cash equivalents or liquid assets 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% of the aggregate principal amount of loans outstanding under the Hercules Loan Agreement, which equals $15.0 million as of the issuance of these consolidated financial statements. The Company must also continue to achieve a performance covenant, which includes maintaining a 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"). Such Performance Covenant is waived during any period in which:

(i.) the Company maintains Qualified Cash, as defined in the Hercules Loan Agreement, in an aggregate amount equal to at least 75% of loans outstanding under the Hercules 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)	Year Ended December 31,		
	2025	2024	2023
Total interest expense	$ 8,876	$ 8,768	$ 5,777
Non-cash interest expense	$ 865	$ 856	$ 929

The annual effective interest rate on the Hercules Loan Agreement as of December 31, 2025 was 11.2%. There were no principal payments due or paid under the Hercules Loan Agreement during the years ended December 31, 2025 and 2024.

As of December 31, 2025, future principal and accrued end-of-term payments of $76.7 million under the Hercules Loan Agreement are due on July 1, 2027.

(in thousands), Year Ending December 31,	Total
2026	—
2027	76,703
Long-term debt, including end-of-term payments	$ 76,703

12. Leases

The Company has a lease agreement for its facilities in Boston, Massachusetts, which is the Company's principal executive offices, and previously had a lease in Vienna, Austria (the "Vienna Lease"), which was previously the Company's research and development center. 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 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.

Vienna Austria Lease— The Company had an operating lease for approximately 1,200 square meters of laboratory and office space in Vienna, Austria that commenced in February 2021 for a term of seven years. Effective November 30, 2025, the Company terminated the Vienna Lease in accordance with a lease amendment entered into with the landlord. Accordingly, the Company accelerated the amortization of the right-of-use asset to zero and adjusted the remaining lease obligation to zero as of December 31, 2025.

The components of lease expense for the years ended December 31, 2025, 2024 and 2023 were as follows:

(dollars in thousands)	Year Ended December 31,		
Lease Cost	**2025**	**2024**	**2023**
Fixed operating lease cost	$ 2,120	$ 1,953	$ 2,084
Total lease expense	$ 2,120	$ 1,953	$ 2,084
Other information			
Sublease income	$ —	$ —	$ 195
Operating cash outflows from operating leases	$ 1,398	$ 1,377	$ 1,385
Weighted-average remaining lease term	0.9 years	2.3 years	3.2 years
Weighted-average discount rate	11.5 %	11.5 %	11.5 %

Maturities of lease liabilities due under lease agreements as of December 31, 2025 are as follows (in thousands):

Maturity of lease liabilities	Operating Leases
2026	$ 1,052
Total lease payments	1,052
Less: interest	(58)
Total operating lease liabilities as of December 31, 2025	$ 994

13. Commitments and Contingencies

The Company has agreements with contract research organizations ("CROs") pursuant to which the Company and the CROs are conducting clinical trials. 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 has agreements with contract manufacturing organizations ("CMOs") for the production of mavorixafor for use in clinical trials and for the commercial supply of XOLREMDI. The Company's agreement with the CMO who produces batches of drug substance for use in the Company's clinical and commercial drug supply contains cancellation provisions that would require the Company to pay up to the full contract value upon cancellation. As of December 31, 2025, the Company has approximately $1.0 million of such commitments in place subject to cancellation provisions.

License Agreements
See Note 4 for a description of licensing 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, 2025 or December 31, 2024.

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 any legal proceedings.

14. Common Stock and Preferred Stock

Common Stock—As of December 31, 2025, the Company's Certificate of Incorporation, as amended and restated, authorizes the Company to issue 500 million shares of common stock, par value $0.001 per share. 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 any preferred stock that may be issued. 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.

Preferred Stock—As of December 31, 2025, the Company's Certificate of Incorporation, as amended, authorizes the Company to issue 10 million shares of $0.001 par value share. As of December 31, 2025 and December 31, 2024, no shares of preferred stock were outstanding.

Reverse Stock Split— On April 17, 2025, the Company's stockholders approved an amendment to the Certificate of Incorporation to combine outstanding shares of the Company's common stock into a lesser number of outstanding shares, by a ratio of not less than one-for-fifteen and not more than one-for-thirty and the Board of Directors approved a Reverse Stock Split at a ratio of 30 for one. The Reverse Stock Split was effective on April 28, 2025. As a result of the Reverse Stock Split, every 30 shares of common stock issued or outstanding were automatically reclassified into one validly issued, fully-paid and non-assessable new share of common stock, subject to the treatment of fractional shares, without any action on the part of the holders. Proportional adjustments were made to the number of shares of common stock awarded and available for issuance under the Company's equity incentive plans, as well as the exercise price and the number of shares issuable upon the exercise or conversion of outstanding stock options and other equity securities. Outstanding warrants were also adjusted in accordance with their terms, which resulted in proportionate adjustments being made to the number of shares issuable upon exercise of such warrants and to the exercise and redemption prices of such warrants. The Reverse Stock Split did not affect the number of authorized shares of common stock or the par value of the common stock. Unless otherwise noted, all references to common stock share and per share amounts in this Annual Report on Form 10-K have been retroactively adjusted to reflect the Reverse Stock Split.

ATM Sales Agreement — The Company is party to a Controlled Equity Offering℠ Sales Agreement ("ATM"), dated as of August 7, 2020, pursuant to which the Company may offer and sell shares of its common stock through one or more investment banks. For the year ended December 31, 2025 and to date through December 31, 2025, the Company has sold $9.7 million and $24.2 million, respectively, of its common stock, net of offering costs, under the ATM. Pursuant to its Registration Statement on Form S-3 that became effective on August 24, 2023 and the related ATM prospectus contained therein, the Company may offer and sell shares of its common stock having an aggregate offering price of up to an additional $65.3 million.

Purchase Agreement— In June 2025, the Company entered into a common stock purchase agreement ("Purchase Agreement") with Lincoln Park Capital Fund LLC pursuant to which Lincoln Park committed to purchase, at the Company's request from time to time over a 24-month period, shares of common stock having an aggregate offering price of up to $40.0 million, subject to certain limitations including a common share cap that will require shareholder approval to exceed (the "Purchase Shares"). In consideration for entering into the Purchase Agreement, the Company issued 137,099 shares of common stock (the "Commitment Shares") to Lincoln Park as a commitment fee. The fair value of the Commitment Shares, which was consideration given to Lincoln Park in exchange for entering into the agreement and was not significant, was measured on the issuance date as determined based on the closing price of the Company's common stock. Through August 21, 2025, the Company sold 1,297,936 shares of common stock to Lincoln Park for aggregate proceeds of $2.2 million. The issuance of the Purchase Shares and Commitment Shares were registered pursuant to an effective shelf registration statement on Form S-3 and

related base prospectus, as supplemented by a prospectus supplement filed on June 23, 2025. The Company terminated the Purchase Agreement effective August 21, 2025.

Q3 2025 Private Placement— On August 11, 2025 and August 12, 2025, the Company entered into securities purchase agreements pursuant to which the Company sold to several institutional and accredited investors (the "Investors") in a private placement (the "Q3 2025 Private Placement"), 11.0 million shares of common stock at a price of $1.42 per share and pre-funded warrants to purchase 48.9 million shares of common stock at a purchase price of $1.419 per pre-funded warrant (representing the price of $1.42 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 Q3 2025 Private Placement closed on August 13, 2025. The net proceeds of $81.0 million, after deducting placement agent fees and offering expenses, received for the shares of common stock and the pre-funded warrants was recorded as permanent equity as the Company concluded that the shares of common stock and pre-funded warrants met the criteria for permanent equity classification.

Also, on August 11, 2025 and August 12, 2025, the Company entered into registration rights agreements with the Investors, pursuant to which the Company agreed to register for resale the common shares issued in the Q3 2025 Private Placement and the issuance of the shares of common stock underlying the pre-funded warrants held by the Investors. Such registration statement was filed on September 10, 2025 and was declared effective by the SEC on September 17, 2025. The Company has agreed to use commercially reasonable efforts to keep such registration statement effective until the date the shares of common stock sold in the Q3 2025 Private Placement and the shares of common stock underlying the pre-funded warrants covered by such registration statement have been sold or may be resold pursuant to Rule 144 without restriction.

Q4 Public Offering—— On October 23, 2025, the Company entered into an underwriting agreement with certain underwriters to issue and sell 45.9 million shares of the Company's common stock at a public offering price of $2.90 per share and, in lieu of common stock to certain investors, pre-funded warrants to purchase 0.7 million shares of the Company's common stock (the "Pre-Funded Warrants") at a public offering price of $2.899 per share, which represents the per share public offering price for the common stock less the $0.001 per share exercise price for each Pre-Funded Warrant (the "Q4 Public Offering"). In addition, the Company granted the underwriters an option for a period of 30 days to purchase up to an additional 7.0 million shares of common stock at the public offering price, less the underwriting discounts and commissions, which the underwriters exercised in full on October 24, 2025. The net proceeds of $145.6 million, after deducting placement agent fees and offering expenses, received for the shares of common stock and the Pre-Funded Warrants was recorded as permanent equity as the Company concluded that the shares of common stock and pre-funded warrants met the criteria for permanent equity classification.

The Pre-Funded Warrants are exercisable at any time after the date of issuance. A holder of Pre-Funded Warrants may not exercise the warrant if the holder, together with its affiliates, would beneficially own more than 4.99% or 9.99%, as applicable, of the number of shares of common stock outstanding immediately after giving effect to such exercise. A holder of Pre-Funded Warrants may increase or decrease this percentage to a percentage not in excess of 19.99% by providing prior notice to the Company, provided that any increase will not be effective until the 61st day after such notice is delivered to the Company.

15. Warrants

In connection with the sale of shares of its common stock, the Company has from time to time issued warrants and pre-funded warrants, which are exercisable for the purchase shares of the Company's common stock. All outstanding warrants and pre-funded warrants are currently exercisable and do not have price reset provisions. Upon the closing of these public and private offerings, the Company received approximately 99% of the exercise price for the pre-funded warrants, for which the remaining exercise price is equal to or less than $0.03 per share.

The following table rolls forward outstanding warrants for the three years ended December 31, 2025.

	Number of Shares	Weighted Average Exercise Price (1)	Weighted Average Contractual Term (Years)
Outstanding and exercisable as of December 31, 2022	3,729,058	$55.80	4.5
Issued	275,438		
Exercised	(249,193)		
Outstanding and exercisable as of December 31, 2023	3,755,303	$56.40	3.5
Expired	(128,893)		
Outstanding and exercisable as of December 31, 2024	3,626,410	$44.36	2.7
Issued	49,552,772		
Exercised	(15,255,271)		
Outstanding and exercisable as of December 31, 2025	37,923,911	$39.18	1.7

(1) Excludes pre-funded warrants, for which the remaining exercise price is between $0.001 and $0.03 per share.

During the year ended December 31, 2025, the Company issued 15,241,246 common shares upon the exercise of 15,255,271 pre-funded warrants pursuant to a net exercise mechanism under the warrants.

As of December 31, 2025, 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	Remaining Exercise Price	Expiration Date
October 25, 2016	171	$ 593.40	October 24, 2026
December 28, 2017	3,863	$ 593.40	December 28, 2027
September 12, 2018	674	$ 593.40	September 12, 2028
October 19, 2018	667	$ 593.40	October 19, 2028
March 13, 2019	166	$ 593.40	March 12, 2029
July 6, 2022	239,401	$ 0.03	n/a
July 6, 2022	1,469,159	$ 32.85	July 6, 2027
December 9, 2022	1,071,248	$ 45.00	December 9, 2027
December 9, 2022	205,000	$ 0.03	n/a
May 18, 2023	165,789	$ 0.03	n/a
August 13, 2025	34,067,773	$ 0.001	n/a
October 27, 2025	700,000	0.001	n/a
	37,923,911		

16. Stock-Based Compensation

Summary of Plans— The Company issues stock awards under the following plans: (a) the Amended and Restated 2017 Equity Incentive Plan (the "2017 Plan"), (b) the Amended and Restated 2017 Employee Stock Purchase Plan (the "2017 ESPP") and (c) 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 of common stock, if any, issued upon the exercise of stock options and vesting of restricted stock units are issued as new shares of common stock from

the share pool reserves established for the applicable plan. 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.

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 January 1, 2026 and 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 a lower amount determined by the Company's Board of Directors. As of December 31, 2025, 0.1 million shares were available for future issuance under the 2017 Plan. As of January 1, 2026, an additional 3.6 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 year ended December 31, 2025, 38,769 shares of common stock were issued under the 2017 ESPP. As of December 31, 2025, approximately 103,102 shares were available for future issuance under the 2017 ESPP.

2019 Inducement Equity Incentive Plan—The 2019 Plan, as amended, is used exclusively for the grant of equity awards to individuals who were not previously employees of the Company, as an inducement material to such individual's entering into employment with the Company. Shares that are expired, forfeited, canceled or otherwise terminated without having been fully exercised become 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, 2025, 1.0 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,		
	2025	2024	2023
Risk-free interest rate	3.9 %	4.1 %	4.1 %
Expected term (in years)	5.8	6.1	6.0
Expected volatility	108.9 %	98.3 %	93.9 %
Expected dividend yield	— %	— %	— %

Stock Options

The following table summarizes the Company's stock option activity for the year ended December 31, 2025:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Contractual Term (Years)	Aggregate Intrinsic Value (in thousands)
Outstanding as of December 31, 2024	394,493	$ 57.09	8.6	$ 269
Granted	12,792,811	1.63		
Forfeited and expired	(1,106,586)	13.65		$ —
Outstanding as of December 31, 2025	12,080,718	$ 2.34	9.6	$ 28,329
Exercisable as of December 31, 2025	77,684	$ 89.58	7.1	$ —
Vested and expected to vest as of December 31, 2025	9,377,359	$ 2.52	9.6	$ 21,890

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. There were no options

exercised in 2025 and 2024. The weighted average grant-date fair value per share of stock options granted during the years ended December 31, 2025, 2024 and 2023 was $1.35, $22.20, and $29.10 respectively.

Restricted Stock Units

The following table summarizes the Company's restricted stock unit activity for the year ended December 31, 2025:

	Number of Shares	Weighted-Average Grant Date Fair Vale
Unvested as of December 31, 2024	198,995	$ 28.87
Granted	278,209	$ 12.65
Vested	(239,924)	$ 21.15
Forfeited	(163,899)	$ 18.70
Unvested as of December 31, 2025	73,381	$ 15.48

The weighted average grant-date fair value per share of restricted stock units granted during the years ended December 31, 2025, 2024 and 2023 was $12.65, $28.50, and $52.80, respectively.

During the year ended December 31, 2025, the Company granted 0.3 million time-based restricted stock units to employees at the grant date fair value of $12.65 per share. These awards vest as the employee provides services to the Company over a three-year vesting period. Also during the year ended December 31, 2025, 0.1 million outstanding performance-based restricted stock units ("PRSUs") vested based on the achievement of an operational milestone. The Company considers the achievement of the remaining operational milestone related to outstanding PRSUs to be probable. Stock-based compensation expense has been recognized for these awards using the accelerated attribution model based on the fair value of the awards as of the date of grant and management's best estimate of the date the probable 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.

During the year ended December 31, 2025, in connection with the termination of employment for three former executives, the vesting of 0.1 million restricted stock units was accelerated resulting in $0.3 million of incremental stock-based compensation expense within general and administrative expense.

Stock-Based Compensation

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

	Year Ended December 31,		
(in thousands)	2025	2024	2023
Research and development expense	$ 1,047	$ 4,291	$ 4,357
General and administrative expense	3,251	3,913	4,330
Total stock-based compensation	$ 4,298	$ 8,204	8,687

As of December 31, 2025, total unrecognized compensation expense related to unvested stock options and restricted stock units was $11.7 million, which is expected to be recognized over a weighted average period of 2.4 years.

Stock Appreciation Rights

The following table rolls forward the Company's stock appreciation right ("SARs") balances and activity as of December 31, 2025. All SARs have been forfeited due to participant terminations as of December 31, 2025.

	Number of Shares
Outstanding as of December 31, 2024	347,068
Forfeited	(347,068)
Outstanding and vested at December 31, 2025	—

17. Income Taxes

During the year ended December 31, 2025, the Company recorded a current global income tax provision of $41 thousand, which primarily relates to its Austrian subsidiary. The Company's overall tax provision for the year ended December 31, 2024 primarily related to U.S. federal income taxes, which was due to a gain on the sale of a priority review voucher that was not fully offset by deductible expenses. The Company's overall tax provision for the year ended December 31, 2023 primarily related to its Austrian subsidiary and Security Corp subsidiary. For the years ended December 31, 2025 and December 31, 2023 in which the Company had taxable losses, the Company recorded no income tax benefits for the net operating losses incurred, research and development credits generated, and orphan drug credits generated in its U.S. entity due to the uncertainty of realizing a benefit from those items.

Loss before the provision for income taxes for the years ended December 31, 2025, 2024 and 2023 consisted of the following:

	Year Ended December 31,		
(in thousands)	2025	2024	2023
United States	$ (79,409)	$ (38,846)	$ (102,126)
Foreign (Austria)	251	1,706	1,037
Loss before provision for income taxes	$ (79,158)	$ (37,140)	$ (101,089)

Income tax expense consisted of the following:

	Year Ended December 31,
(in thousands)	2025
Federal	—
State	13
Foreign (Austria)	28
Total income tax expense	$ 41

Income taxes paid net of refunds received disaggregated by federal, state, and foreign were as follows for the tax year ended (in thousands):

	Year Ended December 31,	
	2025	
Federal	210	
State	90	(a)
Foreign (Austria)	38	
Total income taxes paid	$ 338	

(a) Includes $62 to the state of Pennsylvania

The following table is a reconciliation of the U.S. federal statutory rate of 21% to the Company's effective rate for the year ended December 31, 2025 in accordance with the guidance in ASU 2023-09:

(dollars in thousands)	Year Ended December 31, 2025	
	Amount	Percent
U.S. federal statutory income tax rate	$ (16,623)	21.0 %
State income tax, net of federal benefit (a)	10	— %
Foreign tax effects	(24)	— %
Effect of changes in tax laws or rates enacted in the current period	—	— %
Effect of cross-border tax laws	—	— %
Tax Credits:		
Research and development credits	(2,563)	3.2 %
Orphan drug credits	(3,570)	4.5 %
Changes in valuation allowance	23,440	(29.6)%
Non-taxable or non-deductible items		
Stock-based compensation expense	1,454	(1.8)%
Change in fair value of warrant liability	(2,683)	3.4 %
Other	548	(0.7)%
Changes in unrecognized tax benefits	—	— %
Other adjustments	52	(0.1)%
Effective income tax rate	$ 41	(0.1)%

(a) State tax effects in New Hampshire made up the majority (greater than 50%) of the tax effects in this category.

A reconciliation of the expected income tax expense (benefit) at the U.S. federal statutory income tax rate to the actual income tax expense (benefit) at the Company's effective income tax rate for the years ended December 31, 2024 and 2023 is as follows:

	Year Ended December 31,	
	2024	2023
Expected tax expense (benefit) at U.S. federal statutory income tax rate	(21.0)%	(21.0)%
State income taxes, net of federal benefit	(11.4)	(6.0)
Research and development tax credits	(3.9)	(1.4)
Orphan drug credits	(23.7)	—
Other permanent differences	2.2	(0.2)
Change in deferred tax asset valuation allowance	58.6	29.3
Other	—	(0.7)
Effective income tax rate	0.8 %	— %

Net deferred tax assets as of December 31, 2025 and 2024 consisted of the following:

	December 31,	
(in thousands)	2025	2024
Net operating loss carryforwards	$ 129,943	$ 115,332
Tax credit carryforwards	25,158	18,649
Capitalized research and development expenses	55,625	44,524
Lease liabilities	282	523
Other	3,576	6,658
Total deferred tax assets	214,584	185,686
Valuation allowance	(214,185)	(184,908)
Deferred tax assets, net of valuation allowance	$ 399	$ 778
Right of use assets	399	778
Total deferred tax liabilities	$ 399	$ 778
Total deferred tax assets, net	$ —	$ —

As of December 31, 2025, the Company had U.S. federal and state net operating loss carryforwards of $432.0 million and $383.9 million, respectively, which may be available to offset future taxable income and begin to expire in 2035. The Company has federal net operating losses of $384.0 million, which do not expire, and $48.0 million of federal net operating losses generated prior to 2018 that will expire at various dates through 2037. In addition, as of December 31, 2025, the Company had foreign net operating loss carryforward of $65.3 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, 2025, the Company also had U.S. federal and state research and development tax credit carryforwards of $9.7 million and $3.9 million, respectively, which may be available to offset future tax liabilities and each begin to expire in 2031 and 2030, respectively. Additionally, the Company has U.S. federal Orphan Drug credit carryforwards of $12.4 million which may be available to offset future tax liabilities which begin to expire in 2042.

Utilization of the Company's U.S. net operating loss carryforwards and research and development tax credit carryforwards are subject to a substantial annual limitation under Section 382 of the Internal Revenue Code of 1986 due to ownership changes that have occurred previously and could be further limited as a result of ownership changes that could occur in the future. These ownership changes 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. In 2025, the Company conducted a Section 382 study through December 31, 2024 to assess whether a change or changes of control, as defined by Section 382, have occurred since inception. The Company has determined that multiple changes of control have occurred with the latest in the year ended December 31, 2022. Utilization of the Company's U.S. net operating loss carryforwards generated prior to the last change of control are subject to an annual usage limitation. Net operating loss carryforwards generated after the Tax Cuts and Jobs Act of 2019 ("TCJA") are also subject to an 80% annual usage limitation in addition to the Section 382 limitation. During the year ended December 31, 2025, the Company generated U.S. federal net operating losses of $61.9 million. Given the Company's loss position, the Company has not undertaken an analysis for IRC Section 382 purposes of any activities post December 31, 2024. A full valuation allowance has been provided against the deferred tax assets related to the Company's net operating loss and tax credit carryforwards and, if an adjustment is required, this adjustment would be offset by an adjustment to the valuation allowance.

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, 2025, 2024 and 2023.

Changes in the valuation allowance for deferred tax assets during the years ended December 31, 2025, 2024 and 2023 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)	2025	2024	2023
Valuation allowance, beginning of year	$ (184,908)	$ (163,994)	$ (133,112)
Current year activity	(29,277)	(20,914)	(30,882)
Valuation allowance, end of year	$ (214,185)	$ (184,908)	$ (163,994)

As of December 31, 2025, 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, 2025:

(in millions)	Reserve for Uncertain Tax Position
Balance as of December 31, 2023	$ 0.2
Balance as of December 31, 2024	$ 0.2
Balance as of December 31, 2025	$ 0.2

The Company's U.S. federal and state income tax returns are generally subject to tax examinations for the tax years ended December 31, 2022 through December 31, 2024. 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.

In 2017, the Tax Cuts and Jobs Act of 2017 ("2017 Tax Act") was signed into law. Among other provisions, the 2017 Tax Act requires taxpayers to capitalize and amortize research and experimental ("R&E") expenditures under Section 174 for tax years beginning after December 31, 2021. As such, the rule became effective for the Company during the year ended December 31, 2022 and resulted in the capitalization of R&E costs during the 2024, 2023 and 2022 tax years. Domestic R&E costs were amortized over five years and international R&E costs were amortized over 15 years.

On July 4, 2025, the One Big Beautiful Bill Act ("OBBBA") was enacted. OBBBA, among other changes, makes permanent many of the tax provisions enacted in 2017 as part of the 2017 Tax Act that were set to expire at the end of 2025. The changes include permanently extending 100% bonus depreciation, the immediate deduction for domestic research and experimental expenditures incurred in the current year, and more favorable rules for deducting net business interest offering continued relief for businesses. The Company has incorporated the relevant provisions of OBBBA in its 2025 tax provision and these changes did not have a significant impact to the Company's annual effective tax rate.

18. Net Loss Per Share

Basic and diluted net loss per share was calculated as follows:

	Year Ended December 31,		
(in thousands, except share and per share data)	2025	2024	2023
Numerator:			
Net loss	$ (79,199)	$ (37,450)	$ (101,167)
Denominator:			
Weighted average shares of common stock outstanding—basic and diluted	42,292,818	6,702,073	5,927,082
Net loss per share— basic and diluted	$ (1.87)	$ (5.59)	$ (17.07)

Basic and diluted weighted average shares of common stock outstanding for the years ended December 31, 2025, 2024 and 2023 includes the weighted average effect of pre-funded warrants for the purchase of shares of common stock, for which the remaining unfunded exercise price is less than or equal to $0.30 per share.

For each of the years ended December 31, 2025, 2024, and 2023, during which the Company recorded net loss, the Company's potentially dilutive securities included outstanding stock options, unvested restricted stock units and warrants to purchase shares of common stock. 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 for these periods.

The Company excluded the following potential shares of common stock from the computation of diluted net loss per share for the periods indicated because including them would have had an anti-dilutive effect:

	Year Ended December 31,		
	2025	2024	2023
Options to purchase shares of common stock	12,080,718	394,783	402,690
Unvested restricted stock units	73,381	200,060	265,874
Warrants to purchase shares of common stock (excluding prefunded warrants, which are included in basic shares outstanding)	2,545,948	2,545,960	2,545,960
	14,700,047	3,140,803	3,214,524

19. Gain on Sale of Nonfinancial Asset

In May 2024, the Company entered into contractual arrangement with a third party that transferred the rights to a Priority Review Voucher ("PRV") awarded to the Company as a result of the FDA's approval of XOLREMDI. During the year ended December 31, 2024, the PRV was accounted for as an intangible asset with no accounting cost basis. The third party purchased the PRV for $105.0 million. There were no fees associated with the sale and the Company has no continuing obligations with respect to the PRV. The Company concluded that the third party is "non-customer" as the underlying PRV is not an output of the Company's ordinary commercial activities. Accordingly, the Company accounted for this transaction under ASC Topic 610-20, *Gains and Losses from the Derecognition of Nonfinancial Assets* ("ASC 610-20"). As a result of the transfer of control of the PRV to the third party, the Company derecognized the associated intangible asset and recorded a gain through "gain on sale of non-financial assets." There was no such arrangement for year ended December 31, 2025.

20. Segment Information

The Company's Executive Chairman, who is its 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 hematology diseases. The Company's research, development and commercialization efforts are focused on its lead molecule, mavorixafor.

The measure of profit for the segment is net loss and consisted of the following for the years ended December 31, 2025, 2024, and 2023:

	Year Ended December 31,		
(in thousands)	**2025**	**2024**	**2023**
Revenue from external customers (a)	$ 35,113	$ 2,557	$ —
Compensation expense, excluding stock-based compensation, SARs compensation expense and severance expense	33,283	46,148	28,377
Direct research and development program expenses (mavorixafor)	41,400	41,483	41,163
Gain on sale of non-financial assets	—	(105,000)	—
Other segment items (b)	39,629	57,376	31,627
Net loss (measure of segment profit)	$ (79,199)	$ (37,450)	$ (101,167)

(a) For the year ended December 31, 2025, the Company recognized $6.5 million of revenue from its U.S. customer and $28.6 million of revenue from its customer in the United Kingdom. For the year ended December 31, 2024, the Company recognized $2.6 million of revenue from its U.S. customer and it had no non-U.S. revenue. The Company recognized no revenue for the year ended December 31, 2023.

(b) Other segment items primarily include cost of revenue, non-compensation departmental costs within sales, general and administrative departments, certain unallocated external costs within research and development, stock-based compensation expense, SARs compensation expense, severance expense, other income (expense), and provision for income taxes.

The CODM only receives and reviews information regarding segment assets at the consolidated level. As of December 31, 2025, the Company's single operating segment had long-lived assets, including property and equipment and right-of-use assets, of $1.6 million, all of which were located in the U.S. As of December 31, 2024, the operating segment's long-lived assets were $4.8 million, of which $3.2 million and $1.6 million were located in the U.S. and Austria, respectively.

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Board of Directors

Adam R. Craig, M.D., Ph.D., M.B.A.
Executive Chairman

Michael S. Wyzga
Lead Independent Director

Gary J. Bridger, Ph.D.
Director

Françoise De Craecker
Director

Murray W. Stewart, M.D.
Director

Executive Officers

Adam R. Craig, M.D., Ph.D., M.B.A.
Executive Chairman

John Volpone
President and Chief Operating Officer

David Kirske
Chief Financial Officer, Treasurer and Secretary

Board Committees

Audit Committee
Compensation Committee
Nominating and Corporate Governance Committee

Annual Meeting
The 2026 Annual Meeting of Stockholders will be held virtually 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.

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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."

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Form 10-K Report
The Company's Annual Report on Form 10-K for the year ended December 31, 2025, 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

Investor Contact
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