UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended March 31, 2024

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**

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 is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of May 3, 2024, the registrant had 167,937,781 shares of common stock, $0.001 par value per share, outstanding.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q 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, or the ("Exchange Act"), that relate to future events or to our future operations or financial performance. All statements, other than statements of historical facts, contained in this Quarterly Report on Form 10-Q, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. These statements may be identified by such forward-looking terminology as "may," "should," "expects," "intends," "plans," "anticipates," "believes," "estimates," "predicts," "potential," "continue" or the negative of these terms or other comparable terminology. Our forward-looking statements are based on a series of expectations, assumptions, estimates and projections about our company, are not guarantees of future results or performance and involve substantial risks and uncertainty. We may not actually achieve the plans, intentions or expectations disclosed in these forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in these forward-looking statements. These forward-looking statements are subject to a number of known and unknown risks, uncertainties and assumptions, including risks described in the section titled "Risk Factors" and elsewhere in this report, regarding, among other things:

- the initiation, timing, progress and results of our current and future preclinical studies and clinical trials and related preparatory work and the period during which the results of the trials will become available, as well as our research and development programs;
- the potential benefits, including clinical utility, that may be derived from any of our products or product candidates;
- the timing of and our ability to obtain and maintain regulatory approval of our existing product or product candidates or any product candidates that we may develop in the future, and any related restrictions, limitations, or warnings in the label of any approved product candidates;
- our plans to research, develop, manufacture and commercialize our product or product candidates;
- the timing of our regulatory filings for our product candidates, along with regulatory developments in the United States and other foreign countries;
- the size and growth potential of the markets for our products and product candidates, if approved, and the rate and degree of market acceptance of our products and product candidates, including reimbursement that may be received from payors;
- the benefits of U.S. Food and Drug Administration ("FDA") and European Commission 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 ability to continue as a going concern;
- our plans to in-license, acquire, develop and commercialize additional product candidates;
- the impact of laws and regulations;
- our plans to identify additional product candidates with significant commercial potential that are consistent with our commercial objectives;
- our ability to raise additional capital;

- our strategies, prospects, plans, expectations or objectives; and
- other risks and uncertainties, including those listed under the section titled “Risk Factors” in this Quarterly Report.

You should refer to the section titled “Risk Factors” in this Quarterly 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 Quarterly 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 Quarterly Report.

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 Quarterly Report. Some of the more significant risks include the following:

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

- Our liquidity position raises substantial doubt about our ability to continue as a going concern and we will require substantial additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate any product development programs or commercialization efforts.

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

- We depend almost entirely on the success of our commercial product, XOLREMDI™, 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 on our lead product candidate, mavorixafor, which we are developing for the potential treatment of other chronic neutropenic disorders. We cannot be certain that we will be able to obtain regulatory approval for, or successfully commercialize, mavorixafor for other chronic neutropenic disorders or any other product candidate.

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

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

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

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

- If the commercial opportunity for mavorixafor in WHIM syndrome and other chronic neutropenic disorders is smaller than we anticipate, our potential future revenue from mavorixafor may be adversely affected and our business may suffer.

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

- 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, any product candidate for which we obtain marketing approval could be subject to marketing restrictions or withdrawal from the market and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products.

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

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

- If we are unable to establish 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.

- 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.

- Our commercial products may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives, which would harm our business.

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

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

- Disruptions in our supply chain could delay the commercial launch of our product or product candidates, if approved.

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

- We may depend on collaborations for the development and commercialization of our product candidates. If those collaborations are not successful, we may not be able to capitalize on the market potential of our product candidates.

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

- Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

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

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

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

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

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

PART I FINANCIAL INFORMATION

Item 1. FINANCIAL STATEMENTS.

X4 PHARMACEUTICALS, INC.

CONDENSED CONSOLIDATED BALANCE SHEETS
(In thousands, except share and per share amounts)
(Unaudited)

	March 31, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 60,493	$ 99,216
Marketable securities	20,376	15,000
Research and development incentive receivable	702	562
Prepaid expenses and other current assets	5,762	7,298
Total current assets	87,333	122,076
Property and equipment, net	742	745
Goodwill	17,351	17,351
Right-of-use assets	5,264	5,650
Other assets	1,492	1,436
Total assets	$ 112,182	$ 147,258
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 8,935	$ 8,947
Accrued expenses	13,473	12,816
Current portion of lease liability	1,133	1,099
Total current liabilities	23,541	22,862
Long-term debt, including accretion, net of discount	54,824	54,570
Lease liabilities	2,318	2,612
Warrant liability (Note 4)	29,438	15,683
Other liabilities	1,025	432
Total liabilities	111,146	96,159
Commitments and contingencies (Note 9)		
Stockholders' equity:		
Common stock, $0.001 par value, 500,000,000 shares authorized as of March 31, 2024 and December 31, 2023, respectively; 167,937,781 and 167,434,595 shares issued and outstanding as of March 31, 2024 and December 31, 2023, respectively	168	167
Additional paid-in capital	530,694	528,956
Accumulated other comprehensive loss	(155)	(119)
Accumulated deficit	(529,671)	(477,905)
Total stockholders' equity	1,036	51,099
Total liabilities and stockholders' equity	$ 112,182	$ 147,258

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

X4 PHARMACEUTICALS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(In thousands, except share and per share amounts)

(Unaudited)

	Three Months Ended March 31,	
	2024	**2023**
Operating expenses:		
Research and development	$ 19,854	$ 22,063
Selling, general and administrative	17,435	7,241
Total operating expenses	37,289	29,304
Loss from operations	(37,289)	(29,304)
Other (expense) income, net:		
Interest income	1,066	835
Interest expense	(1,874)	(1,109)
Change in fair value of warrant liability	(13,755)	5,439
Other income, net	105	123
Total other (expense) income, net	(14,458)	5,288
Loss before provision for income taxes	(51,747)	(24,016)
Provision for income taxes	19	4
Net loss	$ (51,766)	$ (24,020)
Net loss per share: basic and diluted	$ (0.26)	$ (0.16)
Weighted average shares of common stock outstanding: basic and diluted	199,991,597	145,967,476
Other comprehensive loss, net of tax:		
Net loss	$ (51,766)	$ (24,020)
Change in unrealized loss related to available-for-sale debt securities	(36)	—
Comprehensive loss	$ (51,802)	$ (24,020)

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

X4 PHARMACEUTICALS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY

(In thousands, except share amounts)

(Unaudited)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2023	167,434,595	$ 167	$ 528,956	$ (119)	$ (477,905)	$ 51,099
Vesting of restricted stock units	503,186	1	(1)			—
Stock-based compensation expense			1,739			1,739
Unrealized loss on marketable securities				(36)		(36)
Net loss					(51,766)	(51,766)
Balance at March 31, 2024	167,937,781	$ 168	$ 530,694	$ (155)	$ (529,671)	$ 1,036

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2022	121,667,250	$ 122	$ 450,786	$ (119)	$ (376,738)	$ 74,051
Vesting of restricted stock units	540,238		—			—
Stock-based compensation			1,645			1,645
Net loss					(24,020)	(24,020)
Balance at March 31, 2023	122,207,488	$ 122	$ 452,431	$ (119)	$ (400,758)	$ 51,676

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

X4 PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(In thousands)
(Unaudited)

	Three Months Ended March 31,	
	2024	**2023**
Cash flows from operating activities:		
Net loss	$ (51,766)	$ (24,020)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	1,739	1,645
Depreciation and amortization expense	62	127
Non-cash lease expense	386	385
Accretion of debt discount	254	225
Change in fair value of warrant liability	13,755	(5,439)
Other	(117)	(51)
Changes in operating assets and liabilities:		
Prepaid expenses, other current assets and research and development incentive receivable	1,062	2,084
Accounts payable	4	(1,714)
Accrued expenses and other long-term liabilities	1,264	496
Lease liabilities	(240)	(250)
Net cash used in operating activities	(33,597)	(26,512)
Cash flows from investing activities:		
Purchase of marketable securities	(10,263)	—
Sales and maturities of marketable securities	5,000	—
Acquisition of property and equipment	(59)	(9)
Net cash used in investing activities	(5,322)	(9)
Cash flows from financing activities:		
Fees paid to amendment loan and security agreement and issuance costs related to the sale of warrants	—	(381)
Repayments of borrowings and accrued end-of-term fees under loan and security agreement	—	(1,300)
Proceeds from sale of common stock, warrants and pre-funded warrants, net of issuance costs	—	(443)
Net cash used in financing activities	—	(2,124)
Effect of exchange rate changes on cash, cash equivalents and restricted cash	(59)	50
Net decrease in cash, cash equivalents and restricted cash	(38,978)	(28,595)
Cash, cash equivalents and restricted cash at beginning of period	100,248	123,028
Cash, cash equivalents and restricted cash at end of period	$ 61,270	$ 94,433

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

1. NATURE OF THE BUSINESS AND BASIS OF PRESENTATION

X4 Pharmaceuticals, Inc. (together with its subsidiaries, the "Company") is a biopharmaceutical company discovering, developing, and commercializing novel therapeutics for the treatment of rare diseases and those with limited treatment options, with a focus on conditions resulting from dysfunction of the immune system. On April 29, 2024, the Company announced that the FDA approved the Company's New Drug Application ("NDA") for mavorixafor, which is being marketed under the trade name XOLREMDI™, for use as an oral, once-daily therapy in patients 12 years of age and older with WHIM syndrome (warts, hypogammaglobulinemia, infections, and myelokathexis), 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 currently engaged in its U.S. launch of XOLREMDI in WHIM syndrome while also planning to seek regulatory approvals to commercialize mavorixafor outside of the U.S. The U.S. approval of XOLREMDI in the WHIM syndrome indication is the first for mavorixafor, which is an orally bioavailable selective antagonist of chemokine receptor CXCR4, a key regulator of the movement of immune cells throughout the body. Due to its ability to increase the mobilization of white blood cells from the bone marrow into the bloodstream, the Company believes that mavorixafor has the potential to provide therapeutic benefit across a variety of immune system disorders in addition to WHIM syndrome. As a result, the Company is conducting a Phase 2 clinical trial evaluating the safety and efficacy of mavorixafor as a monotherapy and in combination with human granulocyte colony-stimulating factor ("G-CSF") in people with certain chronic neutropenic disorders. Interim data from this Phase 2 trial are expected to be presented in June 2024.The Company also plans to initiate a global Phase 3 clinical trial of mavorixafor in the second quarter of 2024 that aims to evaluate the efficacy, safety, and tolerability of oral once-daily mavorixafor with or without G-CSF in people with congenital or acquired primary autoimmune and idiopathic chronic neutropenia who are experiencing recurrent and/or serious infections. The Company is headquartered in Boston, Massachusetts and has a research facility in Vienna, Austria.

***Going Concern Assessment*—**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 condensed consolidated financial statements are issued. Although the Company has an approved drug product, sales of the Company's drug product over the next 12 months will not be sufficient to fund the Company's operating expenses. Since inception, the Company has incurred significant operating losses and negative cash flows from operations. As of March 31, 2024, the Company had $80.9 million of cash, cash equivalents and short-term marketable securities, and an accumulated deficit of $529.7 million. Net cash used in operating activities was $33.6 million for the three months ended March 31, 2024. The Company has a covenant under its Second Amended and Restated Loan and Security Agreement (the "Hercules Loan Agreement") with Hercules Capital Inc. ("Hercules"), that requires that the Company currently maintain a minimum level of cash of $20 million, subject to adjustments beginning January 31, 2025 to 20% of outstanding borrowings. Based on its current cash flow projections, which excludes any new capital raising activities and the potential sale of the Priority Review Voucher that was granted by the FDA concurrent with the approval of XOLREMDI as discussed below, the Company believes it would not maintain the minimum cash required to satisfy this covenant beginning in the first quarter of 2025. In such event, the lender could require the repayment of all outstanding debt. Accordingly, management has concluded that the Company's accumulated deficit, history of losses, future expected losses and negative cash flows met the ASC 205-40 standard for raising substantial doubt about the Company's ability to continue as a going concern. The Company does not have adequate financial resources to fund its forecasted operating costs for at least one year after the date that these consolidated financial statements are issued. The accompanying consolidated financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might result from the outcome of this uncertainty. Accordingly, the consolidated financial statements have been prepared on a basis that assumes the Company will continue as a going concern and which contemplates the realization of assets and satisfaction of liabilities and commitments in the ordinary course of business.

Concurrent with its approval of XOLREMDI and pursuant to its Rare Pediatric Disease designation, the FDA granted the Company a Priority Review Voucher ("PRV") that may be used to obtain Priority Review for a subsequent application or sold to another drug sponsor. The Company's cash flow projections exclude any potential sale of any PRV to a third party and include a $7.0 million milestone payment triggered by the achievement of such approval as discussed in Note 3. To finance its operations in 2025 and beyond, the Company will need to raise additional capital, which cannot be assured. Unless and until the Company reaches profitability in the future, it will require additional capital to fund its operations, which could be raised through a combination of equity offerings, debt financings, other third-party funding, marketing and distribution arrangements and other collaborations and strategic alliances. If the Company is unable to obtain funding, it could be forced to delay, reduce

or eliminate some or all of its research and development programs, product portfolio expansion or commercialization efforts, which would adversely affect its business prospects, or it may be unable to continue operations.

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 the Company's 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.

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

Unaudited Interim Condensed Consolidated Financial Statements— The condensed consolidated balance sheet at December 31, 2023 that is presented in these interim condensed consolidated financial statements was derived from audited financial statements but does not include all disclosures required by accounting principles generally accepted in the United States of America ("GAAP"). The accompanying condensed consolidated financial statements are unaudited. The accompanying unaudited interim condensed consolidated financial statements have been prepared by the Company pursuant to the rules and regulations of the Securities and Exchange Commission ("SEC") for interim financial statements. Accordingly, certain information and footnote disclosures normally included in financial statements prepared in accordance with GAAP have been condensed or omitted pursuant to such rules and regulations. However, the Company believes that the disclosures are adequate to make the information presented not misleading. These unaudited interim condensed consolidated financial statements should be read in conjunction with the Company's audited financial statements and the notes thereto for the year ended December 31, 2023 included in the 2023 Annual Report filed with the SEC on March 21, 2024. In the opinion of management, all adjustments, consisting only of normal recurring adjustments as necessary, for the fair statement of the Company's condensed financial position, condensed results of its operations and comprehensive loss and cash flows have been made. The results of operations for the three months ended March 31, 2024 are not necessarily indicative of the results of operations that may be expected for the year ending December 31, 2024.

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.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Significant Accounting Policies—The Company's significant accounting policies are disclosed in the audited consolidated financial statements and the notes thereto in the Company's Annual Report on Form 10-K for the year ended December 31, 2023 filed with the SEC on March 21, 2024. Since the date of those consolidated financial statements, there have been no material changes to the Company's significant accounting policies.

Restricted Cash

(in thousands)	As of March 31, 2024	As of December 31, 2023
Letter of credit security: Waltham lease	$ —	$ 250
Letter of credit security: Vienna Austria lease	206	211
Letter of credit security: Boston lease	571	571
Total restricted cash	$ 777	$ 1,032
Restricted cash included in prepaid expenses and other current assets	$ —	$ 250
Restricted cash included in other assets	$ 777	$ 782

In connection with the Company's lease agreements for its facilities in Massachusetts and Austria, the Company maintains letters of credit, which are secured by restricted cash, for the benefit of the respective landlord. The Company's Waltham lease agreement expired in December 2023; however, the letter of credit was in place as of December 31, 2023 pending the landlord's completion of its lease expiration procedures. The letter of credit was released in first quarter ended March 31, 2024. In accordance with the Company's Hercules Loan Agreement and as further described in Note 7, the Company at all times must maintain a minimum level of cash of $20.0 million in an account or accounts in which Hercules has a first priority security interest as further described in Note 7.

The following table provides a reconciliation of cash, cash equivalents, and restricted cash reported within the consolidated balance sheets to the sum to the total of amounts shown in the Company's consolidated statements of cash flows as of March 31, 2024 and December 31, 2023:

(in thousands)	March 31, 2024	December 31, 2023
Cash and cash equivalents	$ 60,493	$ 99,216
Restricted cash, current (included within prepaid expenses and other current assets)	—	250
Restricted cash, non-current	777	782
Total cash, cash equivalents and restricted cash	$ 61,270	$ 100,248

Goodwill— There were no triggering events during the three months ended March 31, 2024 that necessitated an interim impairment test of goodwill.

Recently Adopted Accounting Standards

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

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

3. LICENSE, COLLABORATION AND FUNDING AGREEMENTS

Research and Development Incentive Program

The Company participates in a research and development incentive program provided by the Austrian government whereby the Company is entitled to reimbursement by the Austrian government for a percentage of qualifying research and development expenses and capital expenditures incurred by the Company's subsidiary in Austria. As of March 31, 2024, the amount due under the program is $0.7 million, which amount is included in research and development incentive receivable in the condensed consolidated balance sheet. During the three months ended March 31, 2024 and 2023, the Company recorded $0.2 million and $0.1 million of income related to the program within the condensed consolidated statements of operations and comprehensive loss as other income.

License and Collaboration Agreements

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

As of March 31, 2024, the Company is obligated to make future milestone payments in the aggregate amount of up to $20.0 million, contingent upon the achievement by the Company of certain clinical-stage regulatory and sales milestones with respect to licensed products. A $7.0 million regulatory milestone became payable 30 days following the Company's receipt of FDA approval of the Company's NDA on April 26, 2024. The remaining regulatory milestones include (i) $3.0 million for the acceptance by the European Medicines Agency ("EMA") of the Company's first drug application and (ii) $5.0 million upon the notification by the EMA of regulatory approval of the Company's first drug application. The Company must also make one-time sales milestone payments of $0.5 million, $1.5 million and $3.0 million on cumulative net sales of $50.0 million, $150.0 million and $300.0 million, respectively.

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

There were no material modifications of the Company's license or collaboration agreements during the three months ended March 31, 2024.

4. FAIR VALUE OF FINANCIAL ASSETS AND LIABILITIES

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

	Fair Value Measurements as of March 31, 2024 Using:			
(in thousands)	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents—money market funds and U.S. Treasury bills	$ 52,483	$ —	$ —	$ 52,483
Marketable securities— U.S. Treasury notes, U.S. Treasury bills, and federal government agency notes	—	20,376	—	20,376
	$ 52,483	$ 20,376	$ —	$ 72,859
Liabilities:				
Embedded derivative liability	$ —	$ —	$ 10	$ 10
Class C warrant liability (Note 10)	—	—	29,438	29,438
	$ —	$ —	$ 29,448	$ 29,448

	Fair Value Measurements as of December 31, 2023 Using:			
(in thousands)	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents—money market funds and U.S. Treasury bills	$ 76,856	$ 4,985	$ —	$ 81,841
Marketable securities—U.S. Treasury notes, U.S. Treasury bills, and federal government agency notes	—	15,000	—	15,000
	$ 76,856	$ 19,985	$ —	$ 96,841
Liabilities:				
Embedded derivative liability	$ —	$ —	$ 10	$ 10
Class C warrant liability	—	—	15,683	15,683
	$ —	$ —	$ 15,693	$ 15,693

All marketable securities are classified as short-term investments as all are due within one year and include investments in U.S. Treasury notes, U.S. Treasury bills and federal government agency notes. The amortized cost of each investment, individually and in aggregate, approximates fair value. The Company evaluated each marketable security for impairment that is other-than-temporary and concluded that no marketable security was impaired as of March 31, 2024.

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

(in thousands)	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
U.S. Treasury securities	$ 4,173	$ —	$ 2	$ 4,171
Federal Government Agency Securities	16,239	—	34	16,205
Total available-For-sale debt securities	$ 20,412	$ —	$ 36	$ 20,376

The following table provides a roll-forward 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, 2023	$ 10	$ 15,683	$ 15,693
Change in fair value	—	13,755	13,755
Balance as of March 31, 2024	$ 10	$ 29,438	$ 29,448

Valuation of Embedded Derivative Liability— The fair value of the embedded derivative liability recognized in connection with the Company's loan agreement with Hercules (see Note 7), which is associated with additional fees due to Hercules upon events of default, was determined based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy. The fair value of this embedded derivative liability, which is reported within other non-current liabilities on the consolidated balance sheets, is estimated by the Company at each reporting date based, in part, on the results of third-party valuations, which were prepared based on a discounted cash flow model that considered the timing and probability of occurrence of a redemption upon an event of default, the potential amount of prepayment fees or contingent interest upon an event of default and the Company's risk-adjusted discount rate of 17%.

Class C Warrant Liability— In December 2022, the Company issued Class C Warrants for the purchase of shares of its common stock in a public offering. The Class C Warrants are accounted for as a liability on the consolidated balance sheet and are adjusted to fair value at period end through "other (expense) income" on the condensed 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, which represents a Level 3 measurement within the fair value hierarchy, with the following inputs:

	March 31, 2024	December 31, 2023
Common stock price	$1.39	$0.84
Risk-free interest rate	4.3 %	3.9 %
Expected term (in years)	3.7	3.9
Expected volatility	96.1 %	96.2 %
Expected dividend yield	— %	— %

5. PROPERTY AND EQUIPMENT, NET

Property and equipment, net consisted of the following:

(in thousands)	March 31, 2024	December 31, 2023
Leasehold improvements	$ 228	$ 228
Furniture and fixtures	1,289	1,301
Computer equipment	219	160
Software	24	24
Lab equipment	651	651
	2,411	2,364
Less: Accumulated depreciation and amortization	(1,669)	(1,619)
	$ 742	$ 745

Depreciation and amortization expense related to property and equipment was $62 thousand and $127 thousand for the three months ended March 31, 2024 and 2023, respectively.

6. ACCRUED EXPENSES

Accrued expenses consisted of the following:

(in thousands)	March 31, 2024	December 31, 2023
Accrued employee compensation and benefits	$ 7,417	8,195
Accrued external research and development expenses	3,055	2,804
Accrued professional fees	2,374	1,195
Other	627	622
	$ 13,473	$ 12,816

7. LONG-TERM DEBT

Long-term debt consisted of the following:

(in thousands)	March 31, 2024	December 31, 2023
Principal amount of long-term debt	$ 55,000	$ 55,000
Debt discount, net of accretion	(825)	(917)
Cumulative accretion of end of term payments	649	487
Long-term debt	$ 54,824	$ 54,570

Hercules Loan Agreement

The Company entered into a Loan and Security Agreement, as most recently amended, with Hercules Capital, Inc., the ("Hercules Loan Agreement"). The Hercules Loan Agreement provides for an aggregate term loan facility of up to $115.0 million, under which the Company has borrowed an aggregate of $55.0 million of term loans, representing the maximum borrowings allowable as of March 31, 2024. The term loan facility allows for $60.0 million of additional borrowings:

(i) an additional tranche of up to $20.0 million, which became available on April 26, 2024 upon receipt of U.S. approval of XOLREMDI (mavorixafor) in individuals with WHIM syndrome. This tranche is available until September 30, 2024 in the case of the first drawing, and until December 15, 2024 in the case of a second drawing;
(ii) an additional tranche of $7.5 million, which will be available following achievement of a certain clinical development-related milestone through the earlier of (a) 45 days following achievement of such milestone and (b) December 15, 2024; and
(iii) an additional tranche of up to $32.5 million, which will be available subject to approval by Hercules in its sole discretion.

Borrowings under the Hercules Loan Agreement accrue interest at a variable rate equal to the greater of (i) 10.15% or (ii) *The Wall Street Journal* prime rate plus 3.15%. In an event of default and until such event is no longer continuing, the interest rate applicable to borrowings would be increased by 4.0%. Borrowings are repayable in monthly interest-only payments through July 1, 2027, which is the maturity date of the loans. At the Company's option, the Company may prepay all, but not less than all, of the outstanding borrowings, subject to a prepayment premium of 2% during the 12 month period ending January 5, 2025 and 1% thereafter. In addition, the Hercules Loan Agreement provides for payment of end-of-term fees of $2.1 million plus 3.5% of the aggregate principal amount of future loans drawn, if any, payable upon the earlier of maturity or the repayment in full of all obligations under the Hercules Loan Agreement. Borrowings under the Hercules Loan Agreement are collateralized by substantially all of the Company's personal property and other assets except for its intellectual property (but including rights to payment and proceeds from the sale, licensing or disposition of the intellectual property).

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

- On and after January 31, 2025, such amount must equal at least 20% of the aggregate principal amount of loans outstanding under the Hercules Loan Agreement.

- From and after January 31, 2025, the Company must maintain trailing six month net product revenue of at least 55% of its forecast as approved by the Company's Board of Directors (the "Performance Covenant"). However, the Performance Covenant will be waived during any period in which:
  (i) the Company maintains Qualified Cash in an aggregate amount equal to at least 75% of loans outstanding under the Amended Loan Agreement or
  (ii) both (a) the Company maintains a Market Capitalization (as defined in the Hercules Loan Agreement) of at least $450.0 million and (b) the Company maintains Qualified Cash, as defined in the Hercules Loan Agreement, 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)	Three Months Ended March 31,	
	2024	2023
Total interest expense	$ 1,874	$ 884
Non-cash interest expense	$ 254	$ 225

The annual effective interest rate of the Hercules Loan Agreement as of March 31, 2024 is 13.6%. There were no principal payments due or paid under the Hercules Loan Agreement during the three months ended March 31, 2024.

As of March 31, 2024, future principal and accrued end-of-term payments due under the Hercules Loan Agreement were as follows (in thousands):

Year Ending December 31,	Total
2024	$ —
2025	24,720
2026	30,929
Long-term debt	$ 55,649

8. LEASES

The Company has lease agreements for its facilities in Boston, Massachusetts, which is the Company's principal executive office and Vienna, Austria, which is the Company's research and development center. There are no restrictions or financial covenants associated with any of the lease agreements. The Company has an operating lease for approximately 1,200 square meters of laboratory and office space in Vienna, Austria ("Vienna Lease"), which commenced in February 2021 for a term of 7 years. The annual base rent for the Vienna Lease is approximately $282 thousand. The Company also leases approximately 28,000 square feet of office space in Boston, Massachusetts ("Boston Lease"), which serves as the Company's headquarters. Base rental payments are approximately $1.1 million annually, plus certain operating expenses. The term of the Boston Lease will continue until November 2026, unless earlier terminated. The Company has the right to sublease the premises, subject to landlord consent and also has the right to renew the Boston Lease for an additional five years at the then prevailing effective market rental rate. The Company is required to maintain a security deposit in the form of a letter of credit for $0.6 million for the benefit of the landlord.

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

The components of lease expense for the three months ended March 31, 2024 and 2023 were as follows:

(dollars in thousands)	Three Months Ended March 31,	
Lease Cost	**2024**	**2023**
Fixed operating lease cost	$ 489	$ 522
Total lease expense	$ 489	$ 522
Other information		
Operating cash outflows from operating leases	$ 344	$ 346
Sublease income	$ —	$ 49
Weighted-average remaining lease term—operating leases	3.0 years	3.8 years
Weighted-average discount rate—operating leases	11.5 %	11.3 %

Maturities of lease liabilities due under lease agreements that have commenced as of March 31, 2024 are as follows (in thousands):

Maturity of lease liabilities	**Operating Leases**
2024 (remainder of the year)	$ 1,033
2025	1,404
2026	1,334
2027	282
2028	47
Total lease payments	4,100
Less: interest	(649)
Total operating lease liabilities as of March 31, 2024	$ 3,451

9. COMMITMENTS AND CONTINGENCIES

The Company has agreements with clinical research organizations ("CROs") pursuant to which the Company and the CROs are conducting clinical trials. 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. 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 March 31, 2024, the Company has approximately $2.7 million of such commitments in place subject to cancellation provisions.

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 condensed consolidated financial statements as of March 31, 2024 or December 31, 2023.

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.

10. COMMON STOCK AND COMMON STOCK WARRANTS

As of March 31, 2024, the Company's Restated Certificate of Incorporation authorized 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.

Warrants and Pre-Funded Warrants

In connection with public and private sales of shares of its common stock, the Company has 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 $0.01 per share. There were no warrant exercises during the three months ended March 31, 2024.

As of March 31, 2024, the Company's outstanding warrants and pre-funded warrants to purchase shares of common stock consisted of the following:

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

(a) In November 2019, the Company received $11.999 per pre-funded warrant, or $21.0 million in aggregate proceeds. Each pre-funded warrant may be exercised for an additional $0.001 per pre-funded warrant. (b) In March 2021, the Company received $8.69 per pre-funded warrant, or $435 thousand in aggregate proceeds. Each pre-funded warrant may be exercised for an additional $0.01 per pre-funded warrant. (c) In November 2021, the Company received $4.97 per pre-funded warrant, or $10.0 million in aggregate proceeds. Each pre-funded warrant may be exercised for an additional $0.01 per pre-funded warrant. (d) In March 2022, the Company received $1.79 per pre-funded warrant, or $1.4 million in aggregate proceeds. Each pre-funded warrant may be exercised for an additional $0.01 per pre-funded warrant. (e) In July 2022, the Company received $1.094 per pre-funded warrant, or $14.5 million in aggregate proceeds. Each pre-funded warrant may be exercised for an additional $0.001 per pre-funded warrant. (f) In December 2022, the Company received $1.099 per pre-funded warrant, or $7.5 million in aggregate proceeds. (g) In May 2023, the Company received $1.519 per pre-funded warrant, or $12.6 million in aggregate proceeds. Each pre-funded warrant may be exercised for an additional $0.001 per pre-funded warrant.

11. STOCK-BASED COMPENSATION

As of March 31, 2024, there are an aggregate of approximately 4.0 million shares of common stock available for issuance under the Company's equity incentive plans. Approximately 4.9 million shares of common stock remain available for issuance under the 2017 ESPP.

Stock Option Valuation— The following table presents, on a weighted average basis, the assumptions used in the Black-Scholes option-pricing model to determine the grant-date fair value of stock options granted to employees, directors and non-employees.

	Three Months Ended March 31,	
	2024	2023
Risk-free interest rate	4.1 %	3.6 %
Expected term (in years)	6.1	6.0
Expected volatility	95.7 %	90.8 %
Expected dividend yield	0 %	0 %

Stock Options

The following table summarizes the Company's stock option activity for the three months ended March 31, 2024:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Contractual Term (Years)	Aggregate Intrinsic Value (in thousands)
Outstanding as of December 31, 2023	6,008,541	$ 2.97	8.6	$ 24
Granted	2,387,467	0.93		
Forfeited and Expired	(313,170)	2.11		
Outstanding as of March 31, 2024	8,082,838	$ 2.40	8.8	$ 2,123
Exercisable as of March 31, 2024	1,535,408	$ 7.44	6.0	$ 113
Vested and expected to vest as of March 31, 2024	6,136,867	$ 2.79	8.6	$ 1,501

The weighted average grant-date fair value per share of stock options granted during the three months ended March 31, 2024 and 2023 was $0.73 and $0.69, respectively.

Restricted Stock Units— The following table summarizes the Company's restricted stock unit activity for the three months ended March 31, 2024:

	Number of Shares
Unvested as of December 31, 2023	3,118,824
Granted	5,882,459
Vested	(503,186)
Forfeited	(126,511)
Unvested as of March 31, 2024	8,371,586

During the three months ended March 31, 2024, the Company granted performance-based restricted stock units ("PRSUs") to its employees. The PRSUs vest 50% based on the Company's achievement of each of two operational milestones conditioned on the grantee's continued employment with the Company. As of March 31, 2024, neither of the two performance criteria had been met. Stock-based compensation expense has been recognized for awards for which vesting is considered probable 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.

Stock-Based Compensation— As of March 31, 2024, total unrecognized compensation expense related to unvested stock options and restricted stock units was $6.5 million, which is expected to be recognized over a weighted average period of 2.4 years.

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

	Three Months Ended March 31,	
(in thousands)	**2024**	**2023**
Research and development expense	$ 783	$ 831
Selling, general and administrative expense	956	814
Total stock-based compensation	$ 1,739	$ 1,645

Stock Appreciation Rights— On February 13, 2024, (the "Grant Date"), the compensation committee of the Board of Directors approved the grant of stock appreciation rights ("SARs"), pursuant to the 2017 Plan, to the Company's executive officers. The SARs have a measurement price per SAR equal to $0.92, the closing price per share of the Company's common stock on the Grant Date, and each grant of SARs will have a maximum term of ten years from the Grant Date. Unless otherwise determined by the Board of Directors, the SARs will be settled in cash upon exercise. The settlement value will be based on the difference between the closing price of the Company's common stock on the date of settlement less $0.92 multiplied by the number of SARs exercised. The SARs will vest and become exercisable in equal annual installments on the first, second, and third anniversaries of the Grant Date, subject to the recipient remaining an employee of the Company through and including each applicable vesting date.

12. INCOME TAXES

For the three months ended March 31, 2024 and 2023, the Company did not record a U.S. federal or state income tax benefit for the net operating losses incurred and research and development credits generated due to the uncertainty of realizing a benefit from those items and a full valuation allowance is has been applied to the Company's net operating losses and research and development credits as of March 31, 2024 . The income tax provision recorded for the three months ended March 31, 2024 and 2023, primarily related to the Company's Austrian subsidiary and its Security Corporation subsidiary that holds a portion of its investment portfolio.

13. NET LOSS PER SHARE

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

	Three Months Ended March 31,	
(in thousands, except share and per share data)	**2024**	**2023**
Numerator:		
Net loss	$ (51,766)	$ (24,020)
Denominator:		
Weighted average shares of common stock outstanding—basic and diluted	199,991,597	145,967,476
Net loss per share attributable to common stockholders— basic and diluted	$ (0.26)	$ (0.16)

Basic and diluted weighted average shares of common stock outstanding for the three months ended March 31, 2024 and March 31, 2023 include the weighted average effect of outstanding pre-funded warrants for the purchase of shares of common stock for which the remaining unfunded exercise price is $0.01 or less per share. The Company's potentially dilutive securities include outstanding stock options, unvested restricted stock units and warrants to purchase shares of common stock for the three

months ended March 31, 2024 and 2023. All 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 shares of common stock outstanding used to calculate both basic and diluted net loss per share attributable to common stockholders is the same. The Company excluded the following potential shares of common stock, presented based on amounts outstanding at each period end, from the computation of diluted net loss per share attributable to common stockholders for the periods indicated because including them would have had an anti-dilutive effect:

	Three Months Ended March 31,	
	2024	2023
Options to purchase shares of common stock	8,082,838	2,830,300
Unvested restricted stock units	8,371,586	5,835,016
Warrants to purchase shares of common stock (excluding prefunded warrants, which are included in basic shares outstanding)	80,244,959	87,720,773
	96,699,383	96,386,089

Item 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

The following information should be read in conjunction with our unaudited condensed consolidated financial statements and the notes thereto included in this Quarterly Report on Form 10-Q and the audited financial information and the notes thereto included in our Annual Report on Form 10-K, which was filed with the Securities and Exchange Commission ("SEC"), on March 21, 2024, the ("Annual Report"). This discussion and analysis contains forward-looking statements that involve significant risks and uncertainties. Our actual results, performance or experience could differ materially from what is indicated by any forward-looking statement due to various important factors, risks and uncertainties, including, but not limited to, those set forth under "Risk Factors" included elsewhere in this Quarterly Report on Form 10-Q.

Overview

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

On April 29, 2024, we announced that the U.S. FDA approved our NDA for mavorixafor, which is being marketed under the trade name XOLREMDI™, for use as an oral, once-daily therapy in patients aged 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. Concurrent with the U.S. approval of XOLREMDI and pursuant to its Rare Pediatric Disease designation, the FDA granted us a Priority Review Voucher that we intend to sell to another drug sponsor.

We are currently engaged in our U.S. launch of XOLREMDI in WHIM syndrome, and have built out our go-to-market organization, with key hires across commercial and medical functions, increased interactions with key stakeholders and rare disease patient advocacy organizations, and continued our disease-awareness campaign to further the understanding of WHIM syndrome and educate patients and physicians on the importance and benefits of early diagnosis. We have entered into agreements with a third-party logistics organization and a specialty pharmacy to support the distribution of XOLREMDI to patients in the U.S. We are also planning to seek regulatory approvals to commercialize mavorixafor outside of the U.S. We expect to submit an application for regulatory approval of mavorixafor for the treatment of WHIM syndrome to the EMA in late 2024 or early 2025. We are also exploring additional potential opportunities in geographies where we may be able to efficiently leverage our FDA approval.

The U.S. approval of XOLREMDI in the WHIM syndrome indication is the first for mavorixafor, which is an orally active bioavailable selective antagonist of chemokine receptor CXCR4, a key regulator of the movement of immune cells throughout the body. Due to its ability to increase the mobilization of white blood cells from the bone marrow into the bloodstream, we believe that mavorixafor has the potential to provide therapeutic benefit across a variety of immune system disorders in addition to WHIM syndrome.

As a result, we are also currently advancing mavorixafor for the treatment of people with certain chronic neutropenic disorders. Following positive results from a Phase 1b clinical trial of a single dose of mavorixafor in people with idiopathic, cyclic, and congenital chronic neutropenia, we are conducting an ongoing Phase 2 clinical trial evaluating the durability, safety, and tolerability of chronic dosing of once-daily oral mavorixafor with or without concurrent treatment with injectable G-CSF in the same patient population. Preliminary results from the trial showed that the first three participants experienced clinically meaningful increases in absolute neutrophil count ("ANC"). We expect to share additional data from the Phase 2 trial in June 2024. We are also planning to initiate a Phase 3 trial of mavorixafor in the second quarter of 2024 that aims to evaluate the efficacy, safety, and tolerability of oral once-daily mavorixafor (with or without G-CSF) in people with congenital or acquired primary autoimmune and idiopathic chronic neutropenia who are experiencing recurrent and/or serious infections.

We believe that successfully developing and commercializing mavorixafor to provide a new therapeutic option to individuals diagnosed with certain immunodeficiencies has the potential to revolutionize the current treatment landscape, which is principally served by injectable and infused therapies.

Results of Operations

Comparison of the Three Months Ended March 31, 2024 and 2023

The following table summarizes the results of our operations for the three months ended March 31, 2024 and 2023:

	Three Months Ended March 31,		
	2024	2023	Change
(in millions)			
Operating expenses:			
Research and development	$ 20	$ 22	$ (2)
Selling, general and administrative	17	7	10
Total operating expenses	37	29	8
Loss from operations	(37)	(29)	(8)
Total other (expense) income, net	(15)	5	(20)
Loss before provision for income taxes	(52)	(24)	(28)
Net loss	$ (52)	$ (24)	$ (28)

Research and Development Expenses

Research and development expenses consist primarily of costs incurred in connection with the discovery and development of our product candidates, including employee salaries and related expenses, preclinical and clinical development expenses for our product candidates; internal and third-party costs of manufacturing our drug products for use in our preclinical studies and clinical trials and validation batches of our drug substance and drug product. Research and development expenses also include facility, depreciation and other expenses; costs related to compliance with regulatory requirements; and payments made under third-party licensing agreements. We expense research and development costs as incurred.

	Three Months Ended March 31,		
	2024	2023	Change
(in millions)			
Direct research and development expenses by product candidate:			
Mavorixafor	$ 11	$ 15	$ (4)
Unallocated expense	9	7	2
Total research and development expenses	$ 20	$ 22	$ (2)

Research and development expenses decreased by $2.2 million in the three months ended March 31, 2024, as compared to the same period in the prior year. Research and development expenses in the prior period included a $5.0 million in-license fee related to a development milestone under our Genzyme agreement. No similar milestone payments were incurred in the current period. Clinical costs, including third-party costs associated with our pivotal Phase 3 clinical trial of mavorixafor for patients with WHIM syndrome, were lower in the current period due to the completion of this clinical trial in 2023. These decreases in research and development expenses were partially offset by higher compensation costs due to an increase in personnel within our research and development functions.

Selling, General and Administrative Expenses

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

During the quarter ended March 31, 2024, we put in place an experienced sales force to support the launch of XOLREMDI. Those costs and other specific costs related to pre-launch activities contributed to the increase in selling, general and administrative expenses during the current period .Selling, general and administrative expenses increased by approximately $10 million, as compared to the same period in the prior year, primarily driven by:

- an increase of approximately $5 million in compensation costs, which include higher costs related to outstanding Stock Appreciation Rights that are measured at fair value at each period end until exercised, and an increase in sales and

marketing personnel as we build out our sales and marketing infrastructure to support our approved product, XOLREMDI in the U.S,; and
- Approximately $5 million in pre-commercial launch activities, including higher outside consulting fees, legal costs, regulatory activities, marketing strategic initiatives, recruiting, training and IT costs.

We expect selling, general and administrative expenses will grow in the future as we continue to build out our selling, general and administrative functions.

Other Expense, Net

	Three Months Ended March 31,		
	2024	2023	Change
(in millions)			
Interest income	$ 1	$ 1	$ —
Interest expense	(2)	(1)	(1)
Change in fair value of Class C warrant liability	(14)	5	(19)
Total other (expense) income, net	$ (15)	$ 5	$ (20)

Other expense, net, for three months ended March 31, 2024 increased approximately $20.0 million as compared to the same period in the prior year primarily due to an increase in the fair value of our Class C warrants, which are accounted for as a liability at fair value. We value our Class C warrants using the Black-Scholes option pricing model, which includes the market value of our common stock as an input. The market price of shares of our common stock increased during the first quarter of 2024, which was the primary contributor to the increase in the Class C warrant liability and associated expense. These Class C warrants will continue to be measured at fair value and may continue to generate gains or losses each quarter, until they are exercised.

Provision for Income Taxes

We did not record a U.S. federal or state income tax benefit for our losses for the three months ended March 31, 2024 and 2023, respectively, due to our conclusion that a full valuation allowance is required against our U.S. federal and state deferred tax assets. For the three months ended March 31, 2024 and 2023, we recorded income tax expense related to our Austrian subsidiary and for our Security Corporation subsidiary that holds a portion of our investment portfolio and associated interest income.

Liquidity and Capital Resources

Sources of Liquidity

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

ATM Sales Agreement — We have entered into a Controlled Equity Offering℠ Sales Agreement ("ATM Sales Agreement"), with certain investment banks (collectively the "Sales Agents"), pursuant to which we may offer and sell, at our sole discretion through one or more of the Sales Agents, shares of our common stock. To date, we have sold approximately $14.3 million of our common stock, net of offering costs, under the ATM Sales Agreement. 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 $75 million.

LPC Agreement — In January 2022, we entered into a purchase agreement, (the "LPC Agreement") with Lincoln Park Capital Fund LLC ("Lincoln Park"), pursuant to which we have the right to sell to Lincoln Park shares of our common stock, having an aggregate value of up to $50.0 million, subject to certain limitations and conditions, at our request during a 36-month period. The shares of common stock that we may sell under the LPC Agreement are capped at 5.6 million, which amount may be adjusted under certain conditions as defined in the LPC Agreement. In January 2022, we raised $3.0 million from the sale of shares of our common stock through the LPC Agreement.

Public and Private Equity Offerings — Over the past several years we have funded our operations primarily from sales of common stock, warrants and prefunded warrants through both public offerings and private placements.

Hercules Loan Agreement —We have a Loan and Security Agreement, amended from time to time, with Hercules Capital, Inc., the ("Hercules Loan Agreement"). The Hercules Loan Agreement provides for a term loan facility of up to $115.0 million, under which we have borrowed an aggregate of $55.0 million of term loans to date representing the maximum borrowings as of March 31, 2024. The term loan facility allows for $60.0 million in additional borrowings, which includes:

- an additional tranche of up to $20.0 million, which became available on April 26, 2024 in either one or two drawings until June 10, 2024 in the case of the first drawing, and until December 15, 2024 in the case of a second drawing;
- an additional tranche of $7.5 million, which will be available following achievement of a certain clinical development-related milestone through the earlier of (a) 45 days following achievement of such milestone and (b) December 15, 2024; and
- an additional tranche of up to $32.5 million, which will be available subject to approval by Hercules in its sole discretion.

Going Concern— Since our inception, we have incurred significant operating losses and negative cash flows from our operations. As of March 31, 2024, our cash and cash equivalents were $60.5 million, our restricted cash balance was $0.8 million and our investment in marketable securities were $20.4 million. We have a covenant under our Hercules Loan Agreement that currently requires that we maintain a minimum level of cash of $20.0 million through January 31, 2025, subject to subsequent reductions thereafter as further described in Note 7 to our condensed consolidated financial statements. Based on our current cash flow projections, which exclude any benefit from the potential sale of our PRV, no additional borrowings that may become available on Hercules Loan Agreement and with no additional external funding, we believe that we will not be able to maintain the minimum cash required to satisfy this covenant beginning in the first quarter of 2025. In such event, the lenders could require the repayment of all outstanding debt.

Management has concluded that substantial doubt exists about our ability to continue as a going concern for the one-year period following the issuance of our condensed consolidated financial statements for the quarter ended March 31, 2024. To finance our operations, we will need to raise additional capital, which cannot be assured. Unless and until we reach profitability in the future, we will require additional capital to fund our operations, which could be raised through a combination of equity offerings, debt financings, other third-party funding, marketing and distribution arrangements and other collaborations and strategic alliances. If we are unable to obtain funding, we could be forced to delay, reduce or eliminate some or all of our research and development programs, product portfolio expansion or commercialization efforts, which would adversely affect our business prospects, or we may be unable to continue operations.

Cash Flows

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

	Three months ended March 31,	
	2024	2023
	(in millions)	
Net loss	$ (52)	$ (24)
Adjustments to reconcile net loss to net cash used in operating activities	16	(3)
Changes in operating assets and liabilities	2	—
Net cash used in operating activities	(34)	(27)
Net cash used in investing activities	(5)	—
Net cash used in financing activities	—	(2)
Net decrease in cash, cash equivalents and restricted cash	(39)	(29)
Cash, cash equivalents and restricted cash, beginning of period	100	123
Cash, cash equivalents and restricted cash, end of period	$ 61	$ 94

Operating Activities

During the three months ended March 31, 2024, net cash used in operating activities was $34 million, primarily resulting from our net loss of $52 million, adjusted for noncash expenses of $16 million and changes in our operating assets and liabilities of $2 million. Non-cash expenses primarily includes a $14 million loss on the change in fair value of our Class C Warrant liability, stock-based compensation expense, non-cash lease expense and non-cash interest expense. Net cash used in operating activities for the three months ended March 31, 2023 was $27 million, primarily resulting from our net losses of $24 million, adjusted for noncash expenses of $3 million. Net cash used in operating activities increased during the three months ended March 31, 2024

as compared to the same period in the prior year primarily due to the payment of our annual bonuses and increases in our pre-commercialization expenses associated with preparations for the U.S. commercial launch of XOLREMDI.

Investing Activities
During the three months ended March 31, 2024, cash used in investing activities included $5 million of net investments in short-term marketable securities. Investing activities in the prior period were not significant.

Financing Activities
There were no cash used in financing activities during the three months ended March 31, 2024. During the three months ended March 31, 2023, net cash used in financing activities was $2.1 million, consisting primarily of fees paid to Hercules for the amendment and restatement of our Hercules Loan Agreement, including the settlement of a $1 million end-of-term payment.

Funding Requirements
Based on our cash, cash equivalents and marketable securities on hand as of March 31, 2024 and the increases to our borrowing capacity noted above and in Note 7 to the accompanying condensed consolidated financial statements, we believe that our cash, cash equivalents and marketable securities will allow us to fund operations into 2025. However, based on our current financial projections, which exclude the potential sale of our PRV, additional borrowings under our Hercules Loan Agreement that could become available, and additional c we believe we would be in violation of a minimum cash covenant of the Hercules Loan Agreement in the first quarter of 2025. In order to fund operations and satisfy the minimum cash covenant in the Hercules Loan Agreement, we will be required to raise additional capital, which may be through a combination of equity offerings, debt financings, other third-party funding, marketing and distribution arrangements and other collaborations and strategic alliances.

During 2025 and beyond, assuming no changes to our current operational expectations, we expect our expenses to continue to increase in connection with our ongoing activities, particularly as we advance the current and anticipated clinical trials of our product candidates in development. Because of the numerous risks and uncertainties associated with research, development and commercialization of pharmaceutical product candidates, we are unable to estimate the exact amount of our funding requirements. Our short term and long term funding requirements will depend on and could increase significantly as a result of many factors, including:

- the scope, number, initiation, progress, timing, costs, design, duration, any potential delays, and results of clinical trials and nonclinical studies for our current or future product candidates, particularly our Phase 2 clinical trial of mavorixafor for the treatment of 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; and
- 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.

Hercules Loan Agreement
Please see Note 7 to the notes to our condensed consolidated financial statements for a full description of our Hercules Loan Agreement.

Critical Accounting Policies and Significant Judgments and Estimates

Our condensed consolidated financial statements are prepared in accordance with generally accepted accounting principles in the United States. The preparation of our condensed 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 condensed 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.

During the three months ended March 31, 2024, there were no material changes to our critical accounting policies as reported for the year ended December 31, 2023 as part of our Annual Report. In addition, see Note 2 of these condensed consolidated financial statements under the heading "Recently Adopted Accounting Pronouncements" for new accounting pronouncements or changes to the accounting pronouncements during the three months ended March 31, 2024.

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 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

As an SRC, we are not required to provide the information requested by this Item.

Item 4 CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

Management's Evaluation of our 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 March 31, 2024, and have concluded that, based on such evaluation, our disclosure controls and procedures were effective as of March 31, 2024 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.

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 March 31, 2024 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II: OTHER INFORMATION

Item 1. 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 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 Quarterly Report on Form 10-Q, including our unaudited condensed consolidated financial statements and related notes hereto, before deciding to invest in our common stock. The occurrence of any of the following risks could have a material adverse effect on our business, financial condition, results of operations and future growth prospects or cause our actual results to differ materially from those contained in forward-looking statements we have made in this report and those we may make from time to time. In these circumstances, the market price of our common stock could decline and you may lose all or part of your investment. We cannot assure you that any of the events discussed below will not occur.

The risk factors denoted with an "", if any, are newly added or have been materially updated from our Annual Report on Form 10-K for the year ended December 31, 2023.*

Risks Related to Our Financial Position and Need for Additional Capital

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

We are a 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 $101.2 million, $93.9 million and $88.7 million for the years ended December 31, 2023, 2022 and 2021, respectively, and were $51.8 million for the three months ended March 31, 2024. As of March 31, 2024, we had an accumulated deficit of $529.7 million. We have funded our operations to date primarily with proceeds from sales of common stock, warrants, and prefunded warrants for the purchase of our preferred stock and our common stock, sales of preferred stock, proceeds from the issuance of convertible debt, and borrowings under loan and security agreements. We have one product approved for commercial sale, XOLREMDI, upon which we depend almost entirely on to produce revenue. XOLREMDI, which has been approved for WHIM syndrome in the U.S., faces an unknown market size and growth potential and we have not generated any revenue from product sales to date, and we may never generate product revenue or achieve profitability.

We expect to continue to incur significant expenses and increasing operating losses for at least the next several years as we conduct additional clinical trials for our product candidates; continue to discover and develop additional product candidates; acquire or in-license other product candidates and technologies; maintain, expand and protect our intellectual property portfolio; hire additional clinical, scientific and commercial personnel; establish a commercial manufacturing source and secure supply chain capacity sufficient to provide commercial quantities of any product candidates for which we may obtain regulatory approval; seek regulatory approvals for any product candidates that successfully complete clinical trials; establish and grow a sales, marketing and distribution infrastructure to commercialize XOLREMDI and any other products for which we may obtain regulatory approval; and add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts. We may encounter unforeseen expenses, difficulties, complications, delays, and/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:

- outcomes and timing of regulatory reviews, approvals and other actions;
- our ability to manufacture any approved products on commercially reasonable terms;
- our ability to establish and 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 Phase 2 clinical trial of mavorixafor for the treatment of chronic neutropenic disorders;
- our ability to raise sufficient funds to support the development and potential commercialization of our product candidates;
- our ability to market our approved product and obtain marketing approval for our product candidates;
- our ability to establish and maintain licensing, collaboration or similar arrangements on favorable terms and whether and to what extent we retain development or commercialization responsibilities under any new licensing, collaboration or similar arrangement;
- the success of any other business, product or technology that we acquire or in which we invest;
- our ability to maintain, expand and defend the scope of our intellectual property portfolio;
- the number and characteristics of product candidates and programs that we pursue;
- hire additional clinical, regulatory and scientific personnel; and
- incur additional legal, accounting and other expenses associated with operating as a public company.

Although we have obtained marketing approval for, and 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.

Our liquidity position raises substantial doubt about our ability to continue as a going concern and we will require substantial additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate any product development programs or commercialization efforts.

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

Our operations have consumed a large amount of cash since inception. To date, we have funded our operations primarily with proceeds from sales of common stock, warrants and prefunded warrants for the purchase of our preferred stock and our common stock, sales of preferred stock, proceeds from the issuance of convertible debt and borrowings under loan and security agreements. We expect our research and development expenses to increase in future periods as we continue to advance the clinical development of our product candidates and prepare for the launch and commercialization of any product candidates for which we receive regulatory approval, including potentially building our own commercial organization to address the U.S. and certain other markets. In addition, if we obtain marketing approval for any of our product candidates that are not then subject to licensing, collaboration or similar arrangements with third parties, we expect to incur significant commercialization expenses related to product sales, marketing, distribution and manufacturing. Furthermore, we expect to incur additional costs associated with operating as a public company.

As of March 31, 2024, we have cash and cash equivalents of $60.5 million and marketable securities of $20.4 million. We will require additional capital to sustain our operations, and to carry out our business plans, which may include raising funds through public or private equity or debt financings, third-party funding, marketing and distribution arrangements, as well as other collaborations, strategic alliances and licensing arrangements, or any combination of these approaches. While we have successfully raised capital in the past, our ability to raise capital in future periods is not assured. We will also require additional capital to satisfy the covenant under our existing debt facility with Hercules Capital, Inc. and certain affiliated entities (“Hercules”) that requires that we maintain a minimum level of cash of $20.0 million through January 2025, and thereafter,

subject to reductions upon the Company's achievement of certain operational milestones. Based on our current cash flow projections, excluding any gross profit related to the potential sale of our drug and excluding the potential sale of the priority review voucher that we received upon approval of our, and including additional available borrowings under our existing debt facility but excluding additional borrowings or other sources of external financing, we would fail to maintain the minimum cash required to satisfy this covenant as soon as the first quarter of 2025. In such event, the lender could require the repayment of all outstanding debt. Based on the foregoing, we have concluded that substantial doubt exists about our ability to continue as a going concern for a period of at least 12 months from the date of issuance of the financial statements appearing elsewhere in this Quarterly Report. Our financial statements have been prepared assuming that we will continue as a going concern, which contemplates the realization of assets and the settlement of liabilities and commitments in the normal course of business. The financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might result from the outcome of the uncertainty described above. See also the risk factor titled "*Our term loan contains restrictions that limit our flexibility in operating our business*" below.

We cannot be certain that additional funding will be available on acceptable terms, or at all. If we are unable to raise additional capital when needed or in sufficient amounts or on terms acceptable to us, we could be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts of one or more of our product candidates or one or more of our other research and development initiatives. In addition, when we need to secure additional financing, such additional fundraising efforts may divert our management from our day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. Any of these events could significantly harm our business, financial condition and prospects, and our stockholders could lose all or part of their investment in our company.

We also could be required to:

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

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

- the outcome, timing and cost of regulatory reviews, approvals or other actions to meet regulatory requirements established by the 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 scope, number, initiation, progress, timing, costs, design, duration, any potential delays, and results of clinical trials and nonclinical studies for our current or future product candidates;
- the clinical development plans that we establish for these product candidates;
- the number and characteristics of product candidates and programs that we develop or may in-license;
- the cost of filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights covering our product candidates, including any such patent claims and intellectual property rights that we have licensed from Genzyme pursuant to the terms of our license agreement with Genzyme or from other third parties;
- our ability to maintain, expand and defend the scope of our intellectual property portfolio, including the cost of defending intellectual property disputes, including patent infringement actions brought by third parties against us or our product candidates;
- the cost and timing of completion of commercial-scale manufacturing activities with respect to our product candidates;
- our ability to establish and maintain licensing, collaboration or similar arrangements on favorable terms and whether and to what extent we retain development or commercialization responsibilities under any new licensing, collaboration or similar arrangement;
- the cost of establishing sales, marketing and distribution capabilities for any product candidates for which we may receive regulatory approval in regions where we choose to commercialize our products on our own;

- the success of any other business, product or technology that we acquire or in which we invest;
- the costs of acquiring, licensing or investing in businesses, product candidates and technologies;
- our need and ability to hire additional management and scientific and medical personnel;
- market acceptance of our product candidates, to the extent any are approved for commercial sale;
- the effect of competing technological and market developments;
- the costs to operate as a public company; and
- business interruptions resulting from pandemics and public health emergencies, geopolitical actions, including war and terrorism or natural disasters including earthquakes, typhoons, floods and fires.

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

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through public or private equity or debt financings, third-party funding, marketing and distribution arrangements, as well as other collaborations, strategic alliances and licensing arrangements, or any combination of these approaches. Other than our common stock purchase agreement with Lincoln Park Capital Fund LLC ("Lincoln Park"), pursuant to which Lincoln Park is obligated, subject to certain limitations and conditions, to purchase up to a remaining $47.0 million in the aggregate of shares of our common stock, we do not have any committed external sources of funds and may seek to raise additional capital at any time. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a holder of our common stock. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, declaring dividends or other distributions, acquiring or licensing intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business and may result in liens being placed on additional assets such as intellectual property. For example, our debt facility with Hercules contains a minimum cash financial covenant that we project we would be in violation of in the first quarter of 2025 based on our current cash flow projections, assuming we do not raise additional funding. 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.

Concurrent with the U.S. approval of XOLREMDI and, pursuant to its Rare Pediatric Disease designation in the U.S. for WHIM syndrome, the FDA granted us a Priority Review Voucher ("PRV") that may be used to obtain Priority Review for a subsequent application or sold to another drug sponsor. The potential sale of a PRV could generate significant cash proceeds, although no assurance be given as to the nature and magnitude or timing of such as ale, if any, of a PRV.

If we raise additional funds through licensing, collaboration or similar arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research and development programs or product candidates or grant licenses on terms that are not favorable to us. If we are unable to raise additional funds through equity or debt financings or through licensing, collaboration or similar arrangements when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

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

To date, we have not generated revenues from any product sales and cannot predict whether when and if we will be able to generate meaningful revenues from sales of XOLREMDI 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 obtain marketing approval and commercialize our product candidates, including mavorixafor, or other product candidates that we may develop, in-license or acquire in the future. Even if we are able to successfully achieve regulatory approval for these product candidates, we are unable to predict the extent of any future losses and do not know when any of these product candidates will generate revenue for us, if at all. Our ability to generate revenue from XOLREMDI , mavorixafor or any of our current or 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 New Drug Applications to the FDA and obtain regulatory approval for indications for which there is a commercial market;
- complete and submit marketing applications to, and obtain regulatory approval from, foreign regulatory authorities;
- set and obtain a commercially viable price for our products;
- obtain commercial quantities of our products at acceptable cost levels;
- develop a commercial organization capable of sales, marketing and distribution for the products we intend to sell ourselves in the markets in which we have retained commercialization rights;
- find suitable collaborators to help us market, sell and distribute our approved product in other markets; and
- obtain coverage and adequate reimbursement from third-party, including government, payors.

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

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

Changes in estimates regarding fair value of intangible assets may result in an adverse impact on our results of operations.
We test goodwill for impairment annually or more frequently if changes in circumstances or the occurrence of events suggest impairment exists. Any significant change in market conditions, including a sustained decline in our stock price, that indicate a reduction in carrying value may give rise to impairment in the period that the change becomes known. For example, as of December 31, 2021, our market capitalization, measured as the price of our common stock multiplied by shares of common stock outstanding, declined to below the value of our net assets, including goodwill. As a result of the sustained decline in the market price of our common stock, the fair value of our single reporting unit, measured based on our market capitalization as of December 31, 2021, was lower than its carrying value and we concluded that goodwill was impaired. Accordingly, we recorded an impairment charge of $9.8 million to reduce the carrying amount of goodwill to $17.4 million as of December 31, 2021. While we determined that goodwill was not impaired based on our quantitative test as of March 31, 2024, future declines in the market value of our common stock may result in additional impairment charges being recorded.

Risks Related to Development of Our Product Candidates

We depend almost entirely on the success of our commercial product, XOLREMDI ™, which has been approved for use in patients 12 years of age and older with WHIM syndrome in the U.S., and on our lead product candidate, mavorixafor, which we are developing for the potential treatment of other chronic neutropenic disorders. We cannot be certain that we will be able to obtain regulatory approval for, or successfully commercialize, mavorixafor for chronic neutropenic disorders other than WHIM, 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 potentially future product candidates, in combination with other therapies, which could expose us to additional risks.

We may develop mavorixafor, and may develop future product candidates, in combination with one or more currently approved therapies. Even 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.

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 of our clinical trials;
- disagreement with the sufficiency of our clinical trials;
- failure to demonstrate the safety and efficacy of mavorixafor or any other product candidate for its proposed indications;

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

The FDA or a comparable foreign regulatory authority may also require more information, including additional nonclinical or clinical data to support approval, which may delay or prevent approval of our commercialization plans, or cause us to abandon the development program. 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, Beth Israel Deaconess Medical Center, Georgetown University and Dana-Farber Cancer Institute to permit us to use patents and patent applications. Termination of these rights or the failure to comply with obligations under these agreements could materially harm our business and prevent us from developing or commercializing our product candidates.

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

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

- our obligation to pay Genzyme future milestone payments in the aggregate amount of up to $20.0 million, of which $7.0 million becomes payable 30 days following the FDA's approval of our NDA for the marketing and sale of mavorixafor in the U.S., contingent upon our achievement of certain late-stage regulatory and sales milestones with respect to licensed products.
- our obligation to pay Genzyme tiered royalties based on net sales of licensed products that we commercialize under the agreement.
- our obligation to pay Genzyme a certain percentage of cash payments received by us or our affiliates in consideration for the grant of a sublicense under the license granted to us by Genzyme.

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

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

drafting or prosecution of the patents and patent applications licensed to us has been conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents.

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

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

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

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

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

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain any of our license agreements on acceptable terms, we may be unable to successfully develop and commercialize the affected product 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 2 clinical trial of mavorixafor for the treatment of chronic neutropenic disorders. For example, as a result of the COVID-19 pandemic, we experienced delays in clinical trial site activation and slower patient enrollment in our clinical trials of mavorixafor for 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 contract research organizations (“CROs”) and clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- delay or failure in obtaining institutional review board (“IRB”) approval to conduct a clinical trial at each site;
- delays resulting from negative or equivocal findings of the Data Safety Monitoring Board (“DSMB”) if any;
- ambiguous or negative results;
- decision by the FDA, a comparable foreign regulatory authority, or recommendation by a DSMB to suspend or terminate clinical trials at any time for safety issues or for any other reason;
- inadequate supply of drug product for use in nonclinical studies or clinical trials;
- lack of adequate funding to continue the product development program;
- external business disruptions affecting the initiation, patient enrollment, development and operation of our clinical trials, including public health emergencies and unforeseen events such as the war in Ukraine; or
- changes in governmental regulations or requirements.

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

Product candidates may cause undesirable side effects that could delay or prevent their marketing approval, limit the commercial profile of an approved label, or result in significant negative consequences following marketing approval, if any, including marketing withdrawal.
Undesirable side effects caused by any of our product candidates that we may develop or acquire could cause us or the FDA or other regulatory authorities to interrupt, delay or halt our clinical trials and could result in more restrictive labels or the delay or denial of marketing approval by the FDA or other regulatory authorities of such product candidates. Results of our clinical trials could reveal a high and unacceptable severity and prevalence of these or other side effects. In such an event, our trials could be suspended or terminated and the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny approval of our product candidates for any or all targeted indications. In addition, any drug-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims. Any of these occurrences may harm our business, financial condition and prospects significantly.

Further, clinical trials by their nature utilize a sample of the potential patient population. With a limited number of patients, rare and severe side effects of our product candidates may only be uncovered with a significantly larger number of patients exposed to the product candidate. 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. For example, as a result of the COVID-19 pandemic, we previously have experienced a slower enrollment pace in some of our clinical trials.

If we cannot identify patients to participate in our clinical trials or if patients are unwilling to participate in our clinical trials for any reason, including if patients choose to enroll in competitive clinical trials for similar patient populations, the timeline for recruiting patients, conducting studies and obtaining regulatory approval of mavorixafor and any other current or future product candidates that we may develop may be delayed. These delays could result in increased costs, delays in advancing our current or future product candidates, delays in testing the effectiveness of our product candidates or termination of the clinical trials altogether.

We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics to achieve diversity in a trial, to complete our current and future clinical trials in a timely manner. In particular, we are currently evaluating mavorixafor for the treatment of chronic neutropenic disorders, which are rare diseases with limited patient pools from which to draw for clinical trials. The eligibility criteria of our clinical trials will further limit the pool of available trial participants. If we experience difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may be forced to delay, limit or terminate ongoing or planned clinical trials of our product candidates, which would delay our ability to obtain approvals and generate product revenues from any of these product candidates.

If the commercial opportunity for mavorixafor in WHIM syndrome and other chronic neutropenic disorders is smaller than we anticipate, our potential future revenue from mavorixafor for the treatment of any of these diseases may be adversely affected and our business may suffer.
If the size of the commercial opportunities in any of our target indications is smaller than we anticipate, we may not be able to achieve profitability and growth. Our lead clinical candidate, mavorixafor, has been approved by the FDA for use as an oral,
once-daily therapy to increase the number of circulating mature neutrophils and lymphocytes of patients aged 12 years and older with WHIM and is being developed as an oral, once-daily therapy for the potential treatment of other chronic neutropenic disorders. We are currently aware of only a few small available patient registries for WHIM syndrome, and we rely on various estimates and assumptions to estimate the addressable WHIM syndrome population. Based on a broad online survey of physicians to validate current prevalence estimates and additional research using artificial intelligence, which interrogated a database of more than 300 million anonymized patient records that spanned 10 years of insurance claims, we estimate there are up to 3,700 diagnosed and undiagnosed WHIM patients in the United States, many of whom were previously undiagnosed. If the commercial opportunity in any of our target indications, including WHIM syndrome is smaller than we anticipate, whether because our estimates of the addressable patient population prove to be incorrect or for other reasons, our potential future revenue from mavorixafor may be adversely affected and our business may suffer.

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

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

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

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

viewed with caution until the final data are available. Differences between preliminary or interim data and final data could significantly harm our business prospects and may cause the trading price of our common stock to fluctuate significantly.

Risks Related to the Marketing and Commercialization of Our Product Candidates

Our approved product and any future approved products may still face future development and regulatory difficulties and will be subject to extensive post-approval regulatory requirements. Additionally, our approved product and any future approved products 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.

Our approved product and product candidates that receive 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, 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 and our product candidates that receive marketing approval will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMP requirements, quality assurance and corresponding maintenance of records and documents and requirements regarding the distribution of samples to physicians and recordkeeping. 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 candidates 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 product candidates, we could become subject to significant liability, which would materially adversely affect our business and financial condition.

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

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

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

Breakthrough Therapy and Fast Track designations are within the discretion of the FDA. Accordingly, even if we believe that our product candidates meet the criteria for designation, the FDA may disagree and instead determine not to make such designation. The receipt of Breakthrough Therapy designation or Fast Track designation for a product candidate may not result in a faster development process, review or approval compared to products considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify for Breakthrough Therapies or Fast Track designation, the FDA may later decide that a product candidate no longer meets the conditions for the designation and rescind the designation.

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

Regulatory authorities in some jurisdictions, including the United States and European Union, may designate drugs for the treatment or prevention of rare diseases or conditions with relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, the ("Orphan Drug Act"), the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition, which is defined as a patient population of fewer than 200,000 individuals in the United States. We received orphan drug designation from the FDA for mavorixafor for the treatment of WHIM syndrome in October 2018, and from the EMA in July 2019. We also received orphan drug designation in the U.S. for mavorixafor for the treatment of Waldenström's macroglobulinemia in June 2022. If a product with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a seven-year period of marketing exclusivity, which precludes the FDA from approving another marketing application for the same drug for the same indication during that time period with some exceptions. In the European Union, the applicable period is 10 years, during which no marketing authorization may be granted for a similar medicinal product to the authorized orphan product for the same indication. The exclusivity period can be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan designation, including if the drug is sufficiently profitable so that marketing exclusivity is no longer justified. Orphan drug exclusivity may be lost in both the United States and European Union under certain limited situations, such as the inability of the holder of the orphan drug designation to produce sufficient quantities of the drug to meet the needs of patients with the rare disease or condition or for certain other reasons.

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 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 establish 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.***

Although we have built a sales or marketing infrastructure, as an organization we have no experience in the sales, marketing or distribution of pharmaceutical products. To achieve commercial success for our approved product for which we retain sales and marketing responsibilities, we are building a focused sales and marketing infrastructure to sell XOLREMDI ™ (mavorixafor) in the U.S. Although our management team has previous experience with such efforts, there can be no assurance that we will be successful in building these operations. If we are unable to establish adequate sales, marketing and distribution capabilities, we may not be able to generate product revenue and may not become profitable. We will also be competing with many companies that currently have extensive and well-funded sales and marketing operations. If any of our product candidates are approved, we may be unable to compete successfully against these more established companies.

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

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

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

If we enter into arrangements with third parties to perform sales, marketing and distribution services, our product revenue or the profitability of these product revenue to us may be lower than if we were to market and sell any products that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell and market our product

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.

We have obtained FDA approval for mavorixafor for use as an oral, once-daily therapy to increase the number of circulating mature neutrophils and lymphocytes in patients aged 12 years and older with WHIM syndrome, and are developing mavorixafor for potential use in other chronic neutropenic disorders. We are aware of other companies that are developing CXCR4 inhibitors that are in a similar stage of development as mavorixafor, including BioLineRx, Noxxon, Upsher-Smith, Polyphor and Glycomimetics. To our knowledge, there do not appear to be any competitors with programs in development for WHIM syndrome or chronic neutropenia disorders. With respect to chronic neutropenia, filgrastim injections (G-CSF) and two biosimilars (Zarxio and Nivestym) are FDA-approved to reduce the incidence and duration of after effects of severe neutropenia (e.g., fever, infections, oropharyngeal ulcers) in symptomatic patients with congenital neutropenia, cyclic neutropenia or idiopathic neutropenia.

In many diseases, these drugs are administered in combination to enhance efficacy. Some of the currently approved drug therapies are branded and subject to patent protection, and others are available on a generic basis. Many of these approved drugs are well-established therapies and are widely accepted by physicians, patients and third-party payors. Insurers and other third-party payors may also encourage the use of generic products. We expect that if any of our product candidates are approved, they will be priced at a significant premium over competitive generic products. This may make it difficult for us to achieve our business strategy of using our product candidates in combination with existing therapies or replacing existing therapies with our product candidates.

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

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

Even if we obtain and maintain approval for our product candidates from the FDA, we may never obtain approval for our product candidates outside of the United States, which would limit our market opportunities and could harm our business.
Approval of a product candidate in the United States by the FDA does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions, and approval by a foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Even if the FDA grants marketing approval for a product candidate, comparable foreign regulatory authorities also must approve the manufacturing and marketing of the product candidate in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those in the United States, including additional preclinical studies or clinical trials. In many countries

outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for any product candidates, is also subject to approval. Obtaining approval for any future product candidates in the European Union from the European Commission following the opinion of the EMA would be a lengthy and expensive process. Even if a product candidate is approved, the FDA or the European Commission, as the case may be, may limit the indications for which the drug may be marketed, require extensive warnings on the drug labeling or require expensive and time-consuming additional clinical trials or reporting as conditions of approval. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of any future product candidates in certain countries.

If we seek approval to commercialize our product candidates outside of the United States, a variety of risks associated with international operations could harm our business.

If we seek approval of our product candidates outside of the United States, we expect that we will be subject to additional risks in commercialization including:

- different regulatory requirements for approval of therapies in foreign countries;
- reduced protection for intellectual property rights;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- foreign reimbursement, pricing and insurance regimes;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical actions, including war and terrorism, or natural disasters and public health epidemics.

We 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 XOLREMDI or future product candidates, even if those candidates obtain marketing approval.

Our ability to commercialize XOLREMDI™ 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 XOLREMDI 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 XOLREMDI 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 XOLREMDI 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 ™ (mavorixafor) 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 participants;
- significant costs to defend any related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue;
- increased insurance costs; and
- the inability to commercialize any products that we may develop.

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

Risks Related to Government Regulation

Our relationships with customers and third-party payors 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, 2023 entitled "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.

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.

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

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

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

We are also subject to other laws and regulations governing our international operations, including regulations administered by the U.S. government and authorities in the European Union or the United Kingdom, including applicable export control regulations, economic sanctions on countries and persons, customs requirements and currency exchange regulations, which we collectively refer to as Trade Control Laws.

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

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

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

Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years the U.S. government has shut down several times and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical FDA, SEC and other government employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, in our operations as a public company, future government shutdowns could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

Risks Related to Our Dependence on Third Parties

We have no experience manufacturing our product 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.

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. 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 launch of our product or product candidates, if approved.
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.

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

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

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

As of March 31, 2024, we had 116 full-time employees. As our development and commercialization plans and strategies develop, or as a result of any future acquisitions, we will need additional managerial, operational, development, sales, marketing, financial and other resources. Our management, personnel and systems currently in place will not be adequate to support this future growth. Future growth would impose significant added responsibilities on our employees, including:

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

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

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

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

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

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

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

The secure processing, storage, maintenance and transmission of this critical information is vital to our operations and business strategy. Additionally, despite the implementation of security measures, our internal computer systems and those of third parties with which we contract are vulnerable to damage from cyber-attacks, computer viruses, breaches, unauthorized access, interruptions due to employee error or malfeasance or other disruptions, or damage from natural disasters, terrorism, war and telecommunication and electrical failures.

In addition, we have implemented a work model that has enabled substantially all of our employees to periodically work remotely, which may make us more vulnerable to cyberattacks. Any such event could compromise our networks and the information stored there could be accessed by unauthorized parties, publicly disclosed, lost or stolen. We have measures in place that are designed to detect and respond to such security incidents and breaches of privacy and security mandates. Any such access, disclosure or other loss of information could result in legal claims or proceedings, liability under laws that protect the privacy of personal information, government enforcement actions and regulatory penalties. Unauthorized access, loss or dissemination could also disrupt our operations, including our ability to conduct research, development and commercialization activities, process and prepare company financial information, manage various selling, general and administrative aspects of our business and damage our reputation, in addition to possibly requiring substantial expenditures of resources to remedy, any of which could adversely affect our business. The loss of clinical trial data could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. In addition, there can be no assurance that we will promptly detect any such disruption or security breach, if at all. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and our research, development and commercialization efforts could be delayed.

Our ability to use our net operating losses to offset future taxable income may be subject to certain limitations.
Our net operating loss ("NOL") carryforwards could expire unused and be unavailable to offset future tax liabilities because of their limited duration or because of restrictions under U.S. tax law. As of December 31, 2023, we had U.S. federal and state NOLs of $400.0 million and $389.0 million, respectively. Our NOLs generated in tax years ending on or prior to December 31, 2017 are only permitted to be carried forward for 20 years under applicable U.S. tax law. Under the Tax Act, as modified by the CARES Act, our federal NOLs generated in tax years ending after December 31, 2017 may be carried forward indefinitely, but the deductibility of federal NOLs, particularly for tax years beginning after December 31, 2020, may be limited. It is uncertain if and to what extent various states will conform to the Tax Act and the CARES Act.

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

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

In October 2018, we entered into a loan and security agreement, as most recently amended in August 2023, with Hercules, secured by a lien on substantially all of our assets, excluding intellectual property. This loan contains various covenants that limit our ability to engage in specified types of transactions. These covenants limit our ability to, among other things:

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

The covenants also include a requirement that we maintain cash in an aggregate amount greater than or equal to $20 million; provided through January 31, 2025; provided however that on or after January 31, 2025, such amount must equal 20% of the aggregate principal amount of loans outstanding under the loan and security agreement. Based on our current cash, cash equivalents and marketable securities and our cash flow projections, excluding the potential sale of the priority review voucher that we received upon approval of the our NDA noted above, and with no other sources of external financing, we believe that we would not meet the minimum cash described above in the first quarter of 2025. A breach of any of the covenants under the loan and security agreement could result in a default under the loan. Upon the occurrence of an event of default under the loan, the lenders could elect to declare all amounts outstanding, if any, to be immediately due and payable and terminate all commitments to extend further credit. If there are any amounts outstanding that we are unable to repay, the lenders could proceed against the collateral granted to them to secure such indebtedness.

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

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

Risks Related to Ownership of Our Common Stock

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

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

- our ability or the ability of our collaborators to develop product candidates and conduct clinical trials that demonstrate such product candidates are safe and effective;
- our ability or the ability of our collaborators to obtain regulatory approvals for product candidates, and delays or failures to obtain such approvals;
- failure of any our product candidates to demonstrate safety and efficacy, receive regulatory approval and achieve commercial success;
- failure to maintain our existing third-party license, manufacturing and supply agreements;
- failure by us or our licensors to prosecute, maintain or enforce our intellectual property rights;
- changes in laws or regulations applicable to our current or future product candidates;
- any inability to obtain adequate supply of product candidates or the inability to do so at acceptable prices;

- adverse decisions by regulatory authorities;
- introduction of new or competing products by our competitors;
- failure to meet or exceed financial and development projections that we may provide to the public;
- the perception of the pharmaceutical industry by the public, legislatures, regulators and the investment community;
- announcements of significant acquisitions, strategic collaborations, joint ventures or capital commitments by us or our competitors;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain intellectual property protection for our technologies;
- additions or departures of key personnel;
- significant lawsuits, including intellectual property or stockholder litigation;
- announcements by us of material developments in our business, financial condition and/or operations;
- if securities or industry analysts do not publish research or reports about us, or if they issue an adverse or misleading opinions regarding our business and stock;
- changes in the market valuations of similar companies;
- general macroeconomic, political and market conditions and overall fluctuations in the financial markets in the United States and abroad;
- sales of our common stock or our stockholders in the future;
- trading volume of our common stock;
- adverse publicity relating to our markets generally, including with respect to other products and potential products in such markets;
- changes in the structure of health care payment systems;
- period-to-period fluctuations in our financial results; and
- the other factors described in this "Risk Factors" section and elsewhere in this Annual Report

In addition, companies trading in the stock market in general have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies, which has resulted in decreased stock prices for many companies notwithstanding the lack of a fundamental change in their underlying business models or prospects. Broad market and industry factors, including potentially worsening economic conditions and other adverse effects, may negatively affect the market price of our common stock, regardless of our actual operating performance. In the past, following periods of volatility in the market price of a company's securities, stockholders have often instituted class action securities litigation against those companies. Such litigation, if instituted, could result in substantial costs and diversion of management attention and resources, which could significantly harm our business, financial condition, results of operations and reputation.

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

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

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

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

reports on us regularly, demand for our common stock could decrease, which in turn could cause our stock price or trading volume to decline.

We do not anticipate that we will pay any cash dividends in the foreseeable future.
The current expectation is that we will retain our future earnings to fund the development and growth of our business. In addition, the terms of our debt agreements preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain, if any, for the foreseeable future. We are prohibited from declaring or paying any cash dividends under our existing loan and security agreement with Hercules.

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

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

If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results or prevent fraud. As a result, stockholders could lose confidence in our financial and other public reporting, which would harm our business and the trading price of our common stock.
We are subject to the reporting requirements of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), the Sarbanes-Oxley Act of 2002 and the rules and regulations of The Nasdaq Stock Market ("Nasdaq"). Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002 ("Section 404"), we are required to perform system and process evaluation and testing of our internal control over financial reporting to allow our management to report on the effectiveness of our internal control over financial reporting in this Annual Report.

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

Pursuant to Section 404, we are required to furnish a report by our management on our internal control over financial reporting beginning with this Annual Report. However, while we remain a non-accelerated filer, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. When we cease to be a smaller reporting company and no longer qualify as a non-accelerated filer, we will be required to incur substantial additional professional fees and internal costs to expand our accounting and finance functions in order to include such attestation report.

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

We are a "smaller reporting company" and cannot predict if the reduced reporting requirements applicable to smaller reporting companies will make our securities less attractive to investors.

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

We cannot predict if investors will find our securities less attractive because we may rely on the exemptions and reduced disclosure obligations applicable to smaller reporting companies. If some investors find our securities less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

We may become involved in securities class action litigation or shareholder derivative litigation that could divert management's attention and harm our business and insurance coverage may not be sufficient to cover all costs and damages.
In the past, securities class action or shareholder derivative litigation has often followed certain significant business transactions, such as the sale of a business division or announcement of a merger. This risk is especially relevant for us because biopharmaceutical companies have experienced significant stock price volatility in recent years. We may become involved in this type of litigation in the future. The outcome of litigation is necessarily uncertain, and we could be forced to expend significant resources in the defense of such suits, and we may not prevail. Monitoring and defending against legal actions is time-consuming for our management and detracts from management's ability to fully focus our internal resources on our business activities. In addition, we may incur substantial legal fees and costs in connection with any such litigation. We have not established any reserves for any potential liability relating to any such potential lawsuits. It is possible that we could, in the future, incur judgments or enter into settlements of claims for monetary damages. We currently maintain insurance coverage for some of these potential liabilities. Other potential liabilities may not be covered by insurance, insurers may dispute coverage or the amount of insurance may not be enough to cover damages awarded. In addition, certain types of damages may not be covered by insurance, and insurance coverage for all or certain forms of liability may become unavailable or prohibitively expensive in the future. A decision adverse to our interests on one or more legal matters or litigation could result in the payment of substantial damages, or possibly fines, and could have a material adverse effect on our reputation, financial condition and results of operations.

Provisions in our corporate charter documents and under Delaware law could make an acquisition of our company, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.
Provisions in our corporate charter and by-laws may discourage, delay or prevent a merger, acquisition or other change in control of our Company that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares. These provisions also could limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that not all members of the board are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of the board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to the board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize the board of directors to issue preferred stock without stockholder approval, which could be used to institute a shareholder rights plan, or so-called "poison pill," that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by the board of directors; and

- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or by-laws.

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

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

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

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

Item 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS

None.

Item 3. DEFAULTS UPON SENIOR SECURITIES

None.

Item 4. MINE SAFETY DISCLOSURES

Not applicable.

Item 5. OTHER INFORMATION

Rule 10b5-1 Trading Plans

During the three months ended March 31, 2024, none of the Company's directors or officers adopted, materially modified, or terminated any contract, instruction, or written plan for the purchase or sale of Company securities that was intended to satisfy the affirmative defense conditions of Rule 10b5-1(c) or any non-Rule 10b5-1 trading arrangement.

Item 6. EXHIBITS

Exhibit No.	Exhibit Description	Incorporated by Reference to:			
		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	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
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.				
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 Quarterly Report on Form 10-Q 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 Quarterly Report on Form 10-Q, irrespective of any general incorporation language contained in such filing.

SIGNATURES

Pursuant to the requirements 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: May 7, 2024

By: /s/ Paula Ragan, Ph.D.

Paula Ragan, Ph.D.
President and Chief Executive Officer (Principal Executive Officer)

Date: May 7, 2024

By: /s/ Adam S. Mostafa

Adam S. Mostafa
Chief Financial Officer and Treasurer (Principal Financial and Accounting Officer)

Exhibit 31.1

CERTIFICATION

I, Paula Ragan, Ph.D., certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of X4 Pharmaceuticals, Inc.;

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

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

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

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

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

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

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

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

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

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

Date: May 7, 2024

/s/ Paula Ragan, Ph.D.

Paula Ragan, Ph.D.
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION

I, Adam S. Mostafa, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of X4 Pharmaceuticals, Inc.;

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

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

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

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

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

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

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

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

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

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

Date: May 7, 2024

/s/ Adam Mostafa

Adam S. Mostafa
Chief Financial Officer and Treasurer
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATION

Pursuant to the requirement set forth in Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended, (the "Exchange Act") and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. §1350), Paula Ragan, Ph.D., Chief Executive Officer of X4 Pharmaceuticals, Inc. (the "Company"), and Adam S. Mostafa, Chief Financial Officer of the Company, each hereby certifies that, to the best of his or her knowledge:

1. The Company's Quarterly Report on Form 10-Q for the period ended March 31, 2024, to which this Certification is attached as Exhibit 32.1 (the "Quarterly Report"), fully complies with the requirements of Section 13(a) or Section 15(d) of the Exchange Act; and

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

In Witness Whereof, the undersigned have set their hands hereto as of the 7th day of May, 2024.

/s/ Paula Ragan, Ph.D. /s/ Adam Mostafa

Paula Ragan, Ph.D. Adam S. Mostafa

Chief Executive Officer Chief Financial Officer

(Principal Executive Officer) *(Principal Financial Officer and Principal Accounting Officer)*