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 June 30, 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 **(State or other jurisdiction of incorporation or organization)**	**27-3181608** **(I.R.S. Employer Identification No.)**
61 North Beacon Street, 4th Floor **Boston, Massachusetts** **(Address of principal executive offices)**	**02134** **(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 August 5, 2024, the registrant had 168,499,514 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:

- our expectations and goals for commercialization of 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 that XOLREMDI, our one product approved for commercial sale, upon which we depend almost entirely on to produce revenue faces an unknown market size and growth potential and we have not generated significant revenue from product sales to date, and we may never achieve profitability;
- the initiation, timing, progress and results of our current and future preclinical studies and clinical trials and related preparatory work and the period during which the results of the trials will become available, as well as our research and development programs;
- the potential benefits, including clinical utility, that may be derived from 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 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 or achieve sufficient revenue to properly fund our business and operating plan;
- 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 significant revenue from product sales since our inception and we cannot predict whether or 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 commercialize XOLREMDI and to 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. We expect to continue to incur losses for the foreseeable future, and we may never achieve or maintain profitability.

- 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 and on our development product candidate, mavorixafor, which we are advancing 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 limited 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 disrupt the commercial availability of our product.
- 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.

PART I FINANCIAL INFORMATION

Item 1. FINANCIAL STATEMENTS.

X4 PHARMACEUTICALS, INC.

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

	June 30, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 147,218	$ 99,216
Marketable securities	21,536	15,000
Research and development incentive receivable	854	562
Inventory	831	—
Prepaid expenses and other current assets	4,977	7,298
Total current assets	175,416	122,076
Property and equipment, net	808	745
Goodwill	17,351	17,351
Intangible asset, net	10,375	—
Right-of-use assets	4,872	5,650
Other assets	1,789	1,436
Total assets	$ 210,611	$ 147,258
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 7,986	$ 8,947
Accrued expenses (Note 9)	19,744	12,816
Current portion of lease liability	1,172	1,099
Total current liabilities	28,902	22,862
Long-term debt, including accretion, net of discount	75,030	54,570
Lease liabilities	2,026	2,612
Warrant liability (Note 5)	9,223	15,683
Other liabilities	979	432
Total liabilities	116,160	96,159
Commitments and contingencies (Note 12)		
Stockholders' equity:		
Common stock, $0.001 par value, 500,000,000 shares authorized as of June 30, 2024 and December 31, 2023, respectively; 168,196,180 and 167,434,595 shares issued and outstanding as of June 30, 2024 and December 31, 2023, respectively	168	167
Additional paid-in capital	533,280	528,956
Accumulated other comprehensive loss	(159)	(119)
Accumulated deficit	(438,838)	(477,905)
Total stockholders' equity	94,451	51,099
Total liabilities and stockholders' equity	$ 210,611	$ 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 INCOME (LOSS)

(In thousands, except share and per share amounts)

(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Product revenue, net	$ 563	$ —	$ 563	$ —
Costs and operating expenses:				
Cost of revenue	268	—	268	—
Research and development	20,914	15,601	40,768	37,664
Selling, general and administrative	13,278	10,204	30,713	17,445
Gain on sale of non-financial asset (Note 17)	(105,000)	—	(105,000)	—
Total operating (income) expense	(70,540)	25,805	(33,251)	55,109
Income (loss) from operations	71,103	(25,805)	33,814	(55,109)
Other income (expense), net:				
Interest income	1,565	914	2,631	1,749
Interest expense	(2,176)	(1,148)	(4,050)	(2,257)
Change in fair value of warrant liability	20,215	(29,860)	6,460	(24,421)
Other income, net	144	202	249	325
Total other income (expense), net	19,748	(29,892)	5,290	(24,604)
Income (loss) before provision for income taxes	90,851	(55,697)	39,104	(79,713)
Provision for income taxes	18	15	37	19
Net income (loss)	$ 90,833	$ (55,712)	$ 39,067	$ (79,732)
Net income (loss) per share: basic	$ 0.45	$ (0.33)	$ 0.20	$ (0.51)
Weighted average shares of common stock outstanding: basic	200,440,473	168,737,764	200,216,035	157,415,524
Net income (loss) per share: diluted	$ 0.45	$ (0.33)	$ 0.19	$ (0.51)
Weighted average shares of common stock outstanding: diluted	200,801,311	168,737,764	200,455,883	157,415,524
Other comprehensive income (loss), net of tax:				
Net income (loss)	$ 90,833	$ (55,712)	$ 39,067	$ (79,732)
Change in unrealized loss on marketable debt securities	(6)	—	(42)	—
Comprehensive income (loss)	$ 90,827	$ (55,712)	$ 39,025	$ (79,732)

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
Issuance of shares of common stock under employee stock purchase plan	235,068		158			158
Vesting of restricted stock units	23,331					—
Stock-based compensation expense			2,428			2,428
Unrealized loss on marketable securities				(4)		(4)
Net income (loss)					90,833	90,833
Balance at June 30, 2024	168,196,180	$ 168	$ 533,280	$ (159)	$ (438,838)	$ 94,451

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, 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
Issuance of common stock and prefunded warrants in private placement equity transaction, net of issuance costs	34,521,046	35	60,408			60,443
Issuance of common stock under employee stock purchase plan	114,577		175			175
Exercise of stock and warrants	7,476,345	7	8,804			8,811
Vesting of restricted stock units	98,555	—	—			—
Stock-based compensation			2,142			2,142
Net loss					(55,712)	(55,712)
Balance at June 30, 2023	164,418,011	$ 164	$ 523,960	$ (119)	$ (456,470)	$ 67,535

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)

	Six Months Ended June 30,	
	2024	**2023**
Cash flows from operating activities:		
Net income (loss)	$ 39,067	$ (79,732)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	4,167	3,787
Depreciation and amortization expense	256	253
Gain on sale of non-financial asset	(105,000)	—
Non-cash lease expense	778	775
Accretion of debt discount	461	438
Change in fair value of warrant liability	(6,460)	24,421
Other	(281)	(41)
Changes in operating assets and liabilities:		
Inventory	(831)	—
Prepaid expenses, other current assets and research and development incentive receivable	1,398	479
Accounts payable	(944)	(2,815)
Accrued expenses and other long-term liabilities	4,001	5,024
Lease liabilities	(486)	(526)
Net cash used in operating activities	(63,874)	(47,937)
Cash flows from investing activities:		
Acquisition of intangible asset	(7,000)	—
Proceeds from sale of non-financial asset	105,000	—
Purchase of marketable securities	(13,267)	(4,876)
Sales and maturities of marketable securities	7,000	—
Acquisition of property and equipment	(193)	(17)
Net cash provided by (used in) investing activities	91,540	(4,893)
Cash flows from financing activities:		
Proceeds from issuance of shares of common stock under employee stock purchase plan and from exercise of stock options and warrants	159	8,613
Fees paid to amendment loan 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 borrowings under loan and security agreement	20,000	—
Proceeds from sale of common stock, warrants and pre-funded warrants, net of issuance costs	—	60,282
Net cash provided by financing activities	20,159	67,214
Effect of exchange rate changes on cash, cash equivalents and restricted cash	(77)	44
Net increase in cash, cash equivalents and restricted cash	47,748	14,428
Cash, cash equivalents and restricted cash at beginning of period	100,248	123,028
Cash, cash equivalents and restricted cash at end of period	$ 147,996	$ 137,456
Issuance costs not yet paid related to sale of shares of common stock and warrants	$ —	$ 283
Acquisition of intangible assets included in accrued expenses and other liabilities	$ 3,500	$ —

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 in the U.S. 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 completing 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. Positive interim data from this Phase 2 trial were presented in June 2024. The Company initiated a global, pivotal Phase 3 clinical trial of mavorixafor (the 4WARD study) 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—In accordance with the requirements of Accounting Standards Codification ("ASC") 205-40, the Company has evaluated whether there are certain conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date that the 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. During the quarter ended June 30, 2024, the Company sold a Priority Review Voucher (see Note 17) for $105.0 million in cash. As of June 30, 2024, the Company had $168.8 million of cash, cash equivalents and short-term marketable securities, and an accumulated deficit of $438.8 million. Net cash used in operating activities was $63.9 million for the six months ended June 30, 2024.

The Company has a covenant under its Second Amended and Restated Loan and Security Agreement, as amended (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, at which time the Company will be required to maintain a minimum level of cash equal to at least 20% of outstanding borrowings under the Hercules Loan Agreement, the ("Minimum Liquidity Covenant"). The Company has incurred losses and negative cash flows from operations since its inception, and the Company expects to continue to generate operating losses and negative cash flows from operations in the foreseeable future. Based on its current operating plan which includes estimates of anticipated cash inflows from product sales and cash outflows from operating expenses, the Company believes that its existing cash, cash equivalents and short-term marketable securities of $168.8 million as of June 30, 2024 will enable it to fund its planned operating expenses, debt service obligations and capital expenditures at least through the next 12 months from the issuance date of these condensed consolidated financial statements, while the Company observes the Minimum Liquidity Covenant.
To finance its future operations beyond the one-year period following the issuance of these condensed consolidated financial statements, 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, or 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 and six months ended June 30, 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 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 condensed consolidated financial statements include, but are not limited to, the accrual of research and development expenses, the accrual of operational and financial license milestones (Note 3), the accrual of reserves for variable consideration related to product revenue, 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 other than as follows:

Revenue Recognition— The Company records revenue using the guidance of ASC Topic 606, *Revenue from Contracts with Customers* ("ASC 606"), as amended. Upon the approval by the FDA of the sales and marketing of the Company's lead product candidate, revenue related to its sale and distribution is accounted for under ASC 606. The Company recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration that the Company determines it expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements that the Company determines are within the scope of ASC 606, the Company performs the following five steps: (1) identify the customer and contract with the customer; (2) identify the performance obligations in the contract; (3) determine the transaction

price, adjusted for variable consideration resulting from potential returns, rebates, discounts, and down-stream charges; (4) allocate the transaction price to the performance obligations in the contract; and (5) recognize revenue when the Company satisfies its performance obligations, which is upon shipment of the finished product to the customer.

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

As part of the accounting for its contract arrangements, the Company makes significant judgments, primarily related to the estimation of the amount of variable consideration to include in the transaction price upon delivery of the Company's drug product. The variable consideration includes estimates for discounts, product returns, rebates that will be due to U.S. federal and state payors, such as Medicaid, based on agreements that the Company has with these payors who provide medical insurance to the end patient, and estimated co-pay assistance payments for patients who enroll in the Company's patient assistance program. These variable payments are considered a reduction of the Company's transaction price with its customer and must be estimated at the time the Company's product is delivered to the customer. The Company determines the amount of variable consideration by using the expected value method. Net revenue recognized for each period is the amount for which, based on management's estimate, it is probable that a significant reversal of cumulative revenue recognized will not occur. At the end of each subsequent reporting period, the Company re-evaluates these estimates based on new information and actual operational trends and if necessary, adjusts these variable consideration estimates. Any such adjustments are recorded on a cumulative catch-up basis in the period of the adjustment.

Inventory— Prior to receiving approval from the FDA in April 2024 to sell XOLREMDI (mavorixafor) in the United States, the Company expensed all costs incurred related to the manufacture of mavorixafor as research and development expense due to the inherent risks associated with the development of a drug candidate, the uncertainty about the regulatory approval process and the lack of history for the Company in obtaining of regulatory approval of drug candidates. The Company has capitalized inventory-related costs that were incurred subsequent to FDA approval, such as the bottling, labelling, and packaging of drug product, and the acquisition of raw materials for the production of drug substance to be used in commercial drug product. In connection therewith, the Company values inventories at the lower of cost or estimated net realizable value. The Company determines the cost of inventories on a first-in, first-out ("FIFO") basis. Raw materials and work in process include all inventory costs prior to packaging, and labelling, including raw materials and the active pharmaceutical ingredient used in the drug product. Finished goods include packaged and labelled drug products designated for commercial distribution. Clinical drug supplies are expensed to research and development. Raw materials and work in process that may be used for either research and development or commercial sale are classified as inventory until the material is consumed or otherwise allocated for research and development. If the material may only be used for research and development, it is expensed as research and development.

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

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

Restricted Cash

(in thousands)	As of June 30, 2024	As of December 31, 2023
Letter of credit security: Waltham lease	$ —	$ 250
Letter of credit security: Vienna Austria lease	205	211
Letter of credit security: Boston lease	573	571
Total restricted cash	$ 778	$ 1,032
Restricted cash included in prepaid expenses and other current assets	$ —	$ 250
Restricted cash included in other assets	$ 778	$ 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 10, 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 10.

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 June 30, 2024 and December 31, 2023:

(in thousands)	June 30, 2024	December 31, 2023
Cash and cash equivalents	$ 147,218	$ 99,216
Restricted cash, current (included within prepaid expenses and other current assets)	—	250
Restricted cash, non-current	778	782
Total cash, cash equivalents and restricted cash	$ 147,996	$ 100,248

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

The Company assesses qualitative factors to determine whether the existence of events or circumstances would indicate that it is more likely than not that the fair value of the reporting unit is less than its carrying amount. If after assessing the totality of events or circumstances, the Company were to determine that it is more likely than not that the fair value of the reporting unit is less than its carrying amount, then the Company would perform an interim quantitative impairment test, whereby the Company compares the fair value of the reporting unit to its carrying value. If the fair value of the reporting unit exceeds the carrying value of its net assets, goodwill is not impaired, and no further testing is required. If the fair value of the reporting unit is less than its carrying value, the Company measures the amount of impairment loss, if any, as the excess of the carrying value over the fair value of the reporting unit. There were no triggering events during the three months ended June 30, 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 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 June 30, 2024, the amount due under the program is $0.9 million, which amount is included in research and development incentive receivable in the condensed consolidated balance sheet. During each of the six months ended June 30, 2024 and 2023, the Company recorded $0.3 million of income related to the program within the condensed consolidated statements of operations and comprehensive income (loss) as other income.

License 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 June 30, 2024, the Company is obligated to make future milestone payments in the aggregate amount of up to $13.0 million, contingent upon the achievement by the Company of certain clinical-stage regulatory and sales milestones with respect to licensed products. During the three months ended June 30, 2024, a $7.0 million regulatory milestone was made after receipt of FDA approval of the Company's NDA on April 26, 2024. The payment was recorded as a definite-lived intangible asset as discussed in Note 8. 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 has accrued $3.0 million of regulatory milestones and $0.5 million of sales-based milestones as a component of the definite-lived intangible asset, as management has concluded that the achievement of these milestones is probable.

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 includes these royalties in cost of revenue.

There were no material modifications of the Company's license or research and development incentive agreements during the six months ended June 30, 2024.

4. PRODUCT REVENUE, NET

During the three and six months ended June 30, 2024, the Company recorded $0.7 million of gross revenue from the sale of its product to its customer. The Company also recorded an estimate of variable consideration as a provision against gross revenue resulting in net revenue of $0.6 million for the three and six months ended June 30, 2024.

The following table summarizes the activity in each of the product reserve accounts for the six months ended June 30, 2024.

(in thousands)	Rebates and Discounts	Co-Pay Assistance	Product Returns	Total
Beginning balance at December 31, 2023	$ —	$ —	$ —	$ —
Provision related to revenue in the three and six month period ended June 30, 2024	44	35	92	171
Credits and payments made during the period	(21)	(20)	—	(41)
Balance as of June 30, 2024	$ 23	$ 15	$ 92	$ 130

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

The following table provides a rollforward of accounts receivable for the six months ended June 30, 2024. There was no activity in accounts receivable in the six months ended June 30, 2023.

(in thousands)	Accounts Receivable
Beginning balance at December 31, 2023	$ —
Increase in accounts receivable for drug product sales	713
Decrease in accounts receivable for cash collections	(713)
Balance as of June 30, 2024	$ —

5. FAIR VALUE OF FINANCIAL ASSETS AND LIABILITIES

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

	Fair Value Measurements as of June 30, 2024 Using:			
(in thousands)	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents—money market funds and U.S. Treasury bills	$ 106,171	$ 23,845	$ —	$ 130,016
Marketable securities— U.S. Treasury notes, U.S. Treasury bills, and federal government agency notes	—	21,536	—	21,536
	$ 106,171	$ 45,381	$ —	$ 151,552
Liabilities:				
Embedded derivative liability	$ —	$ —	$ 10	$ 10
Class C warrant liability	—	—	9,223	9,223
	$ —	$ —	$ 9,233	$ 9,233

	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 June 30, 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.

The following table provides amortized cost, unrealized gains and losses and the carrying amount of available-for-sale debt marketable securities as of June 30, 2024:

(in thousands)	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
U.S. Treasury securities	$ 2,210	$ —	$ 4	$ 2,206
Federal Government agency securities	19,368	—	38	19,330
Total available-for-sale debt securities	$ 21,578	$ —	$ 42	$ 21,536

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	—	(6,460)	(6,460)
Balance as of June 30, 2024	$ 10	$ 9,223	$ 9,233

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 10), 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 income (expense)" 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:

	June 30, 2024	December 31, 2023
Common stock price	$0.58	$0.84
Risk-free interest rate	4.4 %	3.9 %
Expected term (in years)	3.4	3.9
Expected volatility	100.5 %	96.2 %
Expected dividend yield	— %	— %

6. INVENTORY

Inventory consists of the following:

(in thousands)	June 30, 2024	December 31, 2023
Raw materials	$ 612	—
Finished goods	219	—
Total inventory	$ 831	$ —

7. PROPERTY AND EQUIPMENT, NET

Property and equipment, net consisted of the following:

(in thousands)	June 30, 2024	December 31, 2023
Leasehold improvements	$ 228	$ 228
Furniture and fixtures	1,381	1,301
Computer equipment	263	160
Software	24	24
Lab equipment	651	651
	2,547	2,364
Less: Accumulated depreciation and amortization	(1,739)	(1,619)
	$ 808	$ 745

Depreciation and amortization expense related to property and equipment was $131 thousand and $253 thousand for the six months ended June 30, 2024 and 2023, respectively.

8. INTANGIBLE ASSET, NET

As of June 30, 2024, the Company's definite-lived intangible asset, which totaled $10.4 million, resulted from the capitalization of certain milestone payments made or accrued related to its license agreement for the intellectual property contained in its drug product. The Company amortizes the intangible asset to cost of revenue over the remaining life of the underlying patent protecting the intellectual property through 2038.

As of June 30, 2024, amortization expense for the next five years and beyond is summarized as follows (in thousands):

Year	Amortization expense
2024 (remainder of year)	$ 375
2025	750
2026	750
2027	750
2028	750
Thereafter	7,000
Total	$ 10,375

The Company began amortizing its finite-lived intangible assets in April 2024 over a 14-year period based on the expected patent exclusivity period for XOLREMDI. Amortization expense totaled $0.1 million for the three and six months ended June 30, 2024. Amortization expense is recorded as a component of cost of revenue on the consolidated statements of operations and comprehensive income (loss).

9. ACCRUED EXPENSES

Accrued expenses consisted of the following:

(in thousands)	June 30, 2024	December 31, 2023
Accrued employee compensation and benefits	$ 7,175	8,195
Accrued external research and development expenses	5,439	2,804
Accrued royalty and milestone payments	3,037	—
Accrued professional fees	2,538	1,195
Accrued revenue reserves	130	—
Other	1,425	622
	$ 19,744	$ 12,816

10. LONG-TERM DEBT

Long-term debt consisted of the following:

(in thousands)	June 30, 2024	December 31, 2023
Principal amount of long-term debt	$ 75,000	$ 55,000
Debt discount, net of accretion	(759)	(917)
Cumulative accretion of end of term payments	789	487
Long-term debt	$ 75,030	$ 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 $75.0 million of term loans, representing the maximum borrowings allowable as of June 30, 2024. During the quarter ended June 30, 2024, the Company borrowed an additional $20.0 million term loan, which became available based on the achievement of an operational milestone. The Hercules Loan Agreement allows for $40.0 million of additional borrowings:

(i.) an additional tranche of $7.5 million, which will become 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
(ii.) 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 $2.8 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 June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Total interest expense	$ 2,176	$ 1,148	$ 4,049	$ 2,257
Non-cash interest expense	$ 207	$ 219	$ 461	$ 444

The annual effective interest rate of the Hercules Loan Agreement as of June 30, 2024 is 12.6%. There were no principal payments due or paid under the Hercules Loan Agreement during the three months ended June 30, 2024. During the quarter ended June 30, 2024, the Company borrowed an additional $20.0 million term loan, which became available based on the achievement of an operational milestone.

As of June 30, 2024, future principal and accrued end-of-term payments of $75.8 million under the Hercules Loan Agreement are due on July 1, 2027.

(in thousands), Year Ending December 31,	Total
2024	$ —
2025	—
2026	—
2027	75,789
Long-term debt	$ 75,789

11. LEASES

The Company has lease agreements for its facility 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"), that 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 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 and six months ended June 30, 2024 and 2023 were as follows:

(dollars in thousands)	Three Months Ended June 30,		Six Months Ended June 30,	
Lease Cost	**2024**	**2023**	**2024**	**2023**
Fixed operating lease cost	$ 489	$ 521	$ 978	$ 1,043
Total lease expense	$ 489	$ 521	$ 978	$ 1,043
Other information				
Operating cash outflows from operating leases	$ 343	$ 345	$ 687	$ 691
Sublease income	$ —	$ 48	$ —	$ 97
Weighted-average remaining lease term—operating leases	2.7	3.6	2.7	3.6
Weighted-average discount rate—operating leases	11.5 %	11.4 %	11.5 %	11.4 %

Maturities of lease liabilities due under lease agreements that have commenced as of June 30, 2024 are as follows (in thousands):

Maturity of lease liabilities	**Operating Leases**
2024 (remainder of the year)	$ 688
2025	1,402
2026	1,332
2027	280
2028	47
Total lease payments	3,749
Less: interest	(551)
Total operating lease liabilities as of June 30, 2024	$ 3,198

12. 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 June 30, 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 June 30, 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.

13. COMMON STOCK AND COMMON STOCK WARRANTS

As of June 30, 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 or six months ended June 30, 2024.

As of June 30, 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
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
	108,792,939		

(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.

14. STOCK-BASED COMPENSATION

As of June 30, 2024, there is an aggregate of approximately 2.0 million shares of common stock available for issuance under the Company's equity incentive plans. Approximately 4.7 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 June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Risk-free interest rate	4.4 %	4.0 %	4.2 %	3.8 %
Expected term (in years)	6.1	6.1	6.1	6.0
Expected volatility	96.5 %	93.4 %	96.0 %	92.1 %
Expected dividend yield	0 %	0 %	0 %	0 %

Stock Options

The following table summarizes the Company's stock option activity for the six months ended June 30, 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	4,373,875	1.09		
Forfeited and Expired	(476,118)	2.12		
Outstanding as of June 30, 2024	9,906,298	$ 2.18	8.8	$ —
Exercisable as of June 30, 2024	1,813,063	$ 6.55	6.2	$ —
Vested and expected to vest as of June 30, 2024	7,672,558	$ 2.47	8.6	$ —

The weighted average grant-date fair value per share of stock options granted during the six months ended June 30, 2024 and 2023 was $0.86 and $1.05, respectively.

Restricted Stock Units— The following table summarizes the Company's restricted stock unit activity for the six months ended June 30, 2024:

	Number of Shares
Unvested as of December 31, 2023	3,118,824
Granted	6,242,459
Vested	(829,851)
Forfeited	(313,961)
Unvested as of June 30, 2024	8,217,471

During the six months ended June 30, 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 June 30, 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 June 30, 2024, total unrecognized compensation expense related to unvested stock options and restricted stock units was $8.5 million, which is expected to be recognized over a weighted average period of 2.2 years.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
(in thousands)	2024	2023	2024	2023
Research and development expense	$ 1,182	$ 1,120	$ 1,965	$ 1,951
Selling, general and administrative expense	1,246	1,022	2,202	1,836
Total stock-based compensation	$ 2,428	$ 2,142	$ 4,167	$ 3,787

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.

15. INCOME TAXES

The income tax provision recorded for the three and six months ended June 30, 2024 and 2023 is primarily related to the Company's Austrian subsidiary and its Security Corporation subsidiary that holds a portion of its investment portfolio.

The Company did not record a U.S. federal or state provision or benefit for income taxes in its consolidated statement of operations and comprehensive income (loss) for the three and six month periods ended June 30, 2024 and 2023. For the six months ended June 30, 2024, the Company generated net income and ordinary income. However, as the Company is projecting an ordinary loss before income taxes for the full year and as the Company has a full valuation allowance on its net operating loss carryforwards, the Company has not recorded a U.S. federal or state income tax expense or benefit for the three month and six months ended June 30, 2024.

The Company continues to maintain a valuation allowance against all remaining net deferred tax assets. The Company believes that it is more likely than not that it will not realize a future tax benefit of these attributes as the Company expects to continue to generate operating losses. Ultimate realization of any deferred tax asset is dependent on the Company's ability to generate

sufficient future taxable income in the appropriate tax jurisdiction before the expiration of carryforward periods, if any. The Company will continue to monitor the valuation allowance assessment throughout the year.

16. NET INCOME (LOSS) PER SHARE

Basic and diluted net income (loss) per share was calculated as follow:

	Three Months Ended June 30,		Six Months Ended June 30,	
(in thousands, except share and per share data)	2024	2023	2024	2023
Numerator:				
Net income (loss)	$ 90,833	$ (55,712)	$ 39,067	$ (79,732)
Denominator:				
Weighted average shares of common stock outstanding—basic	200,440,473	168,737,764	200,216,035	157,415,524
Net income (loss) per share— basic	$ 0.45	$ (0.33)	$ 0.20	$ (0.51)
Effective of dilutive securities				
Time-based restricted stock units	244,804	—	163,072	—
Employee stock purchase plan	116,034	—	76,776	—
Dilutive potential common shares	360,838	—	239,848	—
Weighted average shares of common stock outstanding—diluted	200,801,311	168,737,764	200,455,883	157,415,524
Net income (loss) per share— diluted	$ 0.45	$ (0.33)	$ 0.19	$ (0.51)

Basic and diluted weighted average shares of common stock outstanding for the three and six months ended June 30, 2024 and June 30, 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 and six months ended June 30, 2024 and 2023. For the three and six month periods ended June 30, 2023, during which the Company recorded a net loss, 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." For these periods, the weighted average number of shares of common stock outstanding used to calculate both basic and diluted net loss per share of common stock is the same. For the three and six month ended June 30, 2024, the Company reported net income. For these periods, the dilutive effect of outstanding stock options, restricted stock units and warrants were calculated using the treasury stock method, whereby all such awards were assumed to be exercised at the beginning of the period. The hypothetical proceeds from such exercises, including the average unrecognized stock compensation expense for outstanding stock options and restricted stock units, were assumed to be used to purchase outstanding common stock at the average price during the period. The net share impact of dilutive securities was added to the weighted average basic common shares outstanding to calculate weighted average diluted shares outstanding.

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 income (loss) per share for the periods indicated because including them would have had an anti-dilutive effect:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Options to purchase shares of common stock	9,906,298	3,583,186	9,906,298	3,583,186
Unvested restricted stock units	7,800,805	5,859,232	7,800,805	5,859,232
Warrants to purchase shares of common stock (excluding prefunded warrants, which are included in basic shares outstanding)	76,378,805	80,246,459	76,378,805	80,246,459
	94,085,908	89,688,877	94,085,908	89,688,877

17. GAIN ON SALE OF NON-FINANCIAL ASSET

During the quarter ended June 30, 2024, the Company entered into contractual arrangement with a third party that transferred the rights to a Priority Review Voucher ("PRV") awarded to the Company by FDA under its Rare Pediatric Disease program upon the approval of XOLREMDI. The FDA awards PRVs to sponsors of rare pediatric disease product applications that meet certain criteria to encourage development of new drugs and biologics for the prevention and treatment of rare pediatric diseases.The PRV is accounted for as an intangible asset with no accounting cost basis. The third party purchased the PRV for $105.0 million. There were no fees associated with the sale and the Company has no continuing obligations with respect to the PRV. The Company concluded that the third party is "non-customer" as the underlying PRV is not an output of the Company's ordinary commercial activities. Accordingly, the Company accounted for this transaction under ASC Topic 610-20, *Gains and Losses from the Derecognition of Nonfinancial Assets* ("ASC 610-20"). As a result of the transfer of control of the PRV to the third party, the Company derecognized the associated intangible asset and recorded a gain through "gain on transfer of nonfinancial assets."

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 in the U.S. 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 sold to another drug sponsor shortly thereafter.

XOLREMDI Commercial Launch

We are currently engaged in our U.S. launch of XOLREMDI in WHIM syndrome and have built out our go-to-market organization, with all key hires across commercial and medical functions now complete. We have increased our interactions with targeted physicians and rare disease patient advocacy organizations and continued our disease-awareness campaign to further the understanding of WHIM syndrome and to 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 in the U.S., to mitigate barriers to product access, to provide a suite of patient support services to help patients through their treatment journey. We are planning to seek regulatory approvals to commercialize mavorixafor outside of the U.S. by submitting an application for regulatory approval of mavorixafor for the treatment of WHIM syndrome to the European Medicines Agency ("EMA") by 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, 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.

Phase 2 Clinical Trial in Chronic Neutropenia

We continue to advance 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 ("CN"). We are now completing a Phase 2 clinical trial evaluating the durability of effect, safety, and tolerability of chronic dosing of once-daily oral mavorixafor with or without concurrent treatment with injectable granulocyte colony-stimulating factor ("G-CSF") in the same patient population. In June 2024, we announced positive interim clinical data from this Phase 2 clinical trial assessing the efficacy and safety of mavorixafor in the treatment of people with CN. The interim analysis of data from the ongoing six-month study showed that once-daily oral mavorixafor was generally well tolerated and durably increased participants' absolute neutrophil counts ("ANC") both as a monotherapy and in combination with stable doses of G-CSF, the only therapy approved in the U.S. for severe chronic neutropenia. Full data from the Phase 2 CN trial, including data from a treatment group of participants receiving mavorixafor and dose-adjusted G-CSF, are expected to be presented in November 2024.

Phase 3 Clinical Trial in Chronic Neutropenia

We recently initiated our pivotal global, Phase 3 clinical trial, (the "4WARD" study) to evaluate the efficacy, safety, and tolerability of oral, once-daily mavorixafor (with or without stable doses of G-CSF) in people with congenital, acquired primary autoimmune, or idiopathic CN who are experiencing recurrent and/or serious infections. The 52-week trial is a randomized, double-blind, placebo-controlled, multicenter study aiming to enroll 150 participants.

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 and Six Months Ended June 30, 2024 and 2023

The following table summarizes the results of our operations for the three and six months ended June 30, 2024 and 2023:

	Three Months Ended June 30,			Six Months Ended June 30,		
(in millions)	**2024**	**2023**	**Change**	**2024**	**2023**	**Change**
Product revenue, net	$ 0.6	$ —	$ 0.6	$ 0.6	$ —	$ 0.6
Cost and operating expenses:						
Cost of revenue	0.3	—	0.3	0.3	—	0.3
Research and development	20.9	15.6	5.3	40.7	37.7	3.0
Selling, general and administrative	13.3	10.2	3.1	30.7	17.4	13.3
Gain on sale of non-financial asset	(105.0)	—	(105.0)	(105.0)	—	(105.0)
Total operating (income) expenses	(70.5)	25.8	(96.3)	(33.3)	55.1	(88.4)
Income (loss) from operations	71.1	(25.8)	96.9	33.9	(55.1)	89.0
Total other income (expense), net	19.7	(29.9)	49.6	5.3	(24.6)	29.9
Income (loss) before income taxes	90.8	(55.7)	146.5	39.2	(79.7)	118.9
Provision for income taxes	—	—	—	—	—	—
Net income (loss)	$ 90.8	$ (55.7)	$ 146.5	$ 39.2	$ (79.7)	$ 118.9

Product Revenue, Net

Revenue from the sale of our drug product was $0.6 million for the three and six months ended June 30, 2024. There was no product revenue in the prior periods. We sell our approved drug product in the U.S. to a specialty pharmacy that dispenses the product to patients who have been prescribed our drug product by their health-care providers. Our outsourced patient "Hub", which we refer to as X4Connect™, processes in-bound prescriptions, performs an insurance investigation, and enrolls patients on our patient-assistance program. Gross product revenue is recorded based on the amount of drug product delivered to the specialty pharmacy at our wholesale acquisition cost. For the three and six months ended June 30, 2024, such invoiced revenue was $0.7 million. For these periods, revenue was reduced by $0.1 million for distributor discounts, estimated rebates that we may owe to U.S. government payors, future co-pay assistance payments that we may owe for patients who enroll in our patient assistance program, and for potential product returns, resulting in "net" revenue of $0.6 million for these periods. Under our return goods policy, drug product that is within six months of expiration may be returned for a refund less processing fees. As we have just recently launched our first drug product in the U.S, we have no history of returns or downstream rebates or co-pay assistance payments. Therefore, we expect to adjust these estimates quarterly as new information becomes available.

Cost of Revenue

For the three and six months ended June 30, 2024, cost of revenue of $0.3 million, primarily consists of an insignificant amount of drug product direct costs, $0.125 million for the amortization of an intangible asset related to accrued and paid milestone payments associated with our Genzyme license agreement, approximately $0.1 million of accrued sales-based royalty payments due under our Genzyme license agreement, and approximately $0.1 million of costs related to expediting the supply of drug product into the channel shortly following FDA approval. We expect that as we begin to capitalize inventory costs related to the production of our active pharmaceutical ingredient, which is the primary cost of our inventory, cost of revenue will increase per unit. Had we capitalized these research and development costs into inventory, cost of revenue would have been higher by an insignificant amount.

Research and Development Expenses

Research and development expenses consist primarily of costs incurred in connection with the discovery and development of our product candidates. For the three and six months ended June 30, 2024 and 2023, research and development costs were primarily focused on the advancement and commercialization of mavorixafor. Research and development costs include employee salaries and related expenses, preclinical and clinical development expenses, internal and third-party costs of manufacturing our drug products for use in our preclinical studies and clinical trials. Research and development expenses also

include facility, depreciation and other expenses; costs related to compliance with regulatory requirements; and prior to the FDA's approval of our drug product in the U.S., payments made under third-party licensing agreements were charged to research and development expense.

Research and development expenses increased by $5.3 million and $3.1 million in the three and six months ended June 30, 2024, respectively, as compared to the same periods in the prior year. Research and development cost increased in the current quarter primarily due to an increase in clinical operations costs, including CRO fees and pass-through costs, associated with our Phase 2 CN trial and the launch of our Phase 3 CN clinical trial, as well as increased regulatory, medical affairs and quality costs associated with our second quarter product launch and clinical trials. Research and development expenses were also higher in the current three and six-month periods due to an increase in personnel within our research and development functions. Such increase in the six month period ended June 30, 2024 was partially offset by a $5.0 million in-license fee related to a development milestone under our Genzyme agreement that was changed to research and development expense in 2023 where no such similar charges to research and development expense were recorded in the six months ended June 30, 2024.

Selling, General and Administrative Expenses

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

Selling, general and administrative expenses increased by approximately $3.1 million and $13.3 million in the three and six months ended June 30, 2024, respectively, as compared to the same three month and six month periods in the prior year, respectively, primarily due to an increase in our sales and marketing expenses, which increased by $4.1 million and $9.0 million in the three and six month periods ended June 30, 2024, respectively, as compared to the prior period, as we built out our sales and marketing infrastructure to support the pre-launch and post-launch activities of our approved product, XOLREMDI in the U.S. Costs related to commercial launch activities contributed to the increase in selling, general and administrative expenses during the current three and six-month period. Selling, general and administrative costs also increased in the current six months ended June 30, 2024 as compared to the same period in the prior year due to increases in legal costs, outside consulting fees, recruiting and information technology costs. Selling, general and administrative expenses include compensation expenses for stock appreciation rights ("SARs") granted to our executive officers. We measure these SARs at fair value primarily based on our closing stock price, which may continue to be volatile.

We expect selling, general and administrative expenses will remain relatively consistent for the remainder of the current fiscal year.

Gain on Sale of Non-Financial Asset

During the quarter ended June 30, 2024, we sold a Priority Review Voucher ("PRV") awarded to us as a result of the FDA's approval of XOLREMDI to a third party for $105.0 million in cash. There were no fees associated with the sale. We do not expect to receive or sell a similar PRV in the foreseeable future.

Other Income (Expense), Net

	Three Months Ended June 30,			Six Months Ended June 30,		
(in millions)	2024	2023	Change	2024	2023	Change
Interest income	$ 1.6	$ 0.9	$ 0.7	$ 2.6	$ 1.7	$ 0.9
Interest expense	(2.2)	(1.1)	(1.1)	(4.0)	(2.2)	(1.8)
Change in fair value of Class C warrant liability	20.2	(29.9)	50.1	6.5	(24.4)	30.9
Other income, net	0.1	0.2	(0.1)	0.2	0.3	(0.1)
Total other income (expense), net	$ 19.7	$ (29.9)	$ 49.6	$ 5.3	$ (24.6)	$ 29.9

Other income (expense), net, for three and six months ended June 30, 2024 increased $49.6 million and $29.9 million, respectively, as compared to the same period in the prior year primarily due to a significant decrease in the fair value of our Class C warrants, which are accounted for as a liability at fair value, relative to the prior period. We value these 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 decreased during the second quarter of 2024, which was the primary contributor to

the decrease 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

The income tax provision recorded for the three and six months ended June 30, 2024 and 2023 is primarily related to our Austrian subsidiary and our Security Corporation subsidiary that throughout the year holds a portion of our investment portfolio. We did not record a U.S. federal or state provision or benefit for income taxes for the three and six month period ended June 30, 2024 as, although we generated net income and ordinary income for these periods, we are projecting an ordinary loss before income taxes for the full year and we continue to have a full valuation allowance on our net operating loss carryforwards ("NOLs"). We anticipate that we may generate U.S. and state taxable income for the year ended December 31, 2024 that will be either fully or partially offset by our operating loss carryforwards, subject to an annual limitation pursuant to Section 382 of the Internal Revenue Code. For the three and six month period ended June 30, 2023, we did not record an income tax benefit for our losses as we have a full valuation allowance on our NOL carryforwards.

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 $75.0 million of term loans to date, including $20.0 million in new borrowings during the three months ended June 30, 2024, representing the maximum borrowings as of June 30, 2024. The term loan facility allows for $40.0 million in additional borrowings, which includes:

- 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— We have evaluated whether there are certain conditions and events, considered in the aggregate, that raise substantial doubt about the our ability to continue as a going concern within one year after the date that the condensed consolidated financial statements are issued. Although we have an approved drug product, sales of the our drug product over the next 12 months will not be sufficient to fund our operating expenses. Since inception, we have incurred significant operating losses and negative cash flows from operations. During the quarter ended June 30, 2024, we sold a PRV for $105.0 million in cash. As of June 30, 2024, we had $168.8 million of cash, cash equivalents and short-term marketable securities, and an accumulated deficit of $438.8 million. Net cash used in operating activities was $63.9 million for the six months ended June 30, 2024.

We have a covenant under our Hercules Loan Agreement that requires that we currently maintain a minimum level of cash of $20 million, subject to adjustments beginning January 31, 2025, at which time we will be required to maintain a minimum level of cash equal to at least 20% of outstanding borrowings under the Hercules Loan Agreement, the ("Minimum Liquidity Covenant"). We have incurred losses and negative cash flows from operations since its inception, and we expect to continue to generate operating losses and negative cash flows from operations in the foreseeable future. Based on our current operating plan which includes estimates of anticipated cash inflows from product sales and cash outflows from operating expenses, we believe that our existing cash, cash equivalents and short-term marketable securities of $168.8 million as of June 30, 2024 will enable it to fund its planned operating expenses, debt service obligations and capital expenditures at least through the next 12 months from the issuance date of these condensed consolidated financial statements, while we observe the Minimum Liquidity Covenant.

To finance its future operations beyond the one-year period following the issuance of these condensed consolidated financial statements, 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, or other collaborations and strategic alliances. If we are unable to obtain funding, we could be forced to delay, reduce, or eliminate some or all of our research and development programs, product portfolio expansion or commercialization efforts, which would adversely affect our business prospects, or we may be unable to continue operations.

Cash Flows

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

	Six months ended June 30,	
	2024	2023
	(in millions)	
Net income (loss)	$ 39	$ (80)
Adjustments to reconcile net income (loss) to net cash used in operating activities	(106)	30
Changes in operating assets and liabilities	3	2
Net cash used in operating activities	(64)	(48)
Net cash provided by (used in) investing activities	92	(5)
Net cash provided by financing activities	20	67
Net increase in cash, cash equivalents and restricted cash	48	14
Cash, cash equivalents and restricted cash, beginning of period	100	123
Cash, cash equivalents and restricted cash, end of period	$ 148	$ 137

Operating Activities

During the six months ended June 30, 2024, net cash used in operating activities was $64 million, primarily resulting from operating expenses of $71 million, adjusted for noncash expenses of $5 million and changes in our operating assets and liabilities of $3 million. Non-cash expenses primarily include stock-based compensation expense, non-cash lease expense and non-cash interest expense. Net cash used in operating activities for the six months ended June 30, 2023 was $48 million, primarily resulting from our net losses of $80 million, adjusted for noncash expenses of $30 million. Net cash used in operating activities increased during the six months ended June 30, 2024 as compared to the same period in the prior year primarily due to increases in our sales and marketing and commercialization expenses associated with the U.S. commercial launch of XOLREMDI.

Investing Activities

During the six months ended June 30, 2024, cash provided by investing activities included $105.0 million in proceeds from the sale of a priority review voucher obtained as a result of the approval of our drug product by the FDA. Cash outflows from investing activities included $7.0 million in license payments classified as the acquisition of an intangible assets and $6 million of net investments in short-term marketable securities. During the six months ended June 30, 2023, cash used in investing activities included an investment in short-term marketable debt securities.

Financing Activities
Cash provided by financing activities for the six months ended June 30, 2024 included $20.0 million of new borrowings on our loan facility. During the six months ended June 30, 2023, net cash provided by financing activities was $67.2 million, consisting primarily of net proceeds from a private placement equity offering that provided net proceeds of $60.7 million.

Funding Requirements
Based on our cash, cash equivalents and marketable securities on hand as of June 30, 2024 and the increases to our borrowing capacity noted above and in Note 10 to the accompanying condensed consolidated financial statements, we believe that our cash, cash equivalents and marketable securities will allow us to fund operations for at least the next 12 months. In order to fund operations and satisfy the minimum cash covenant in the Hercules Loan Agreement thereafter, 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, or collaborations and strategic alliances.

During 2025 and beyond, assuming no changes to our current operational expectations, we expect our expenses to be relatively consistent with our current operating expenses excluding the sale of non-financial assets. Because of the numerous risks and uncertainties associated with the future sale of our approved drug product and the research, development, and commercialization of future product candidates, we are unable to estimate the exact amount of our funding requirements. Our short-term and long-term funding requirements will depend on and could increase significantly as a result of many factors, including:

- the scope, number, initiation, progress, timing, costs, design, duration, any potential delays, and results of clinical trials and nonclinical studies for our current or future product candidates, particularly our Phase 3 clinical trial of mavorixafor for the treatment of individuals with chronic neutropenic disorders;
- the outcome, timing and cost of regulatory reviews, approvals or other actions to meet regulatory requirements established by the FDA and comparable foreign regulatory authorities, including the potential for the FDA or comparable foreign regulatory authorities to require that we perform more studies for our product candidates than those that we currently expect;
- our ability to obtain marketing approval for our product candidates;
- the cost of filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights covering our product and product candidates, including any such patent claims and intellectual property rights that we have licensed from Genzyme pursuant to the terms of our license agreement with Genzyme; 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 10 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 June 30, 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 other than as updated in Note 2 of these condensed consolidated financial statements. Also see Note 2 under the heading “Recently Adopted Accounting Pronouncements” for new accounting pronouncements or changes to the accounting pronouncements during the three months ended June 30, 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 June 30, 2024, and have concluded that, based on such evaluation, our disclosure controls and procedures were effective as of June 30, 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 June 30, 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 significant revenue from product sales since our inception. We expect to continue to incur losses for the foreseeable future and we may never achieve or maintain profitability.

We are a commercial-stage biopharmaceutical company. Investment in biopharmaceutical product development is highly speculative because it entails substantial upfront capital expenditures and significant risk that any potential product candidate will fail to demonstrate adequate effect or an acceptable safety profile, gain regulatory approval, become commercially viable, or maintain commercial viability. Since inception, other than during the three and six months ended June 30, 2024, we have incurred significant operating losses. Our net losses were $101.2 million, $93.9 million and $88.7 million for the years ended December 31, 2023, 2022 and 2021, respectively, and we expect to generate a net loss and negative operating cash flows in 2024. As of June 30, 2024, we had an accumulated deficit of $438.8 million. To date, 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 significant revenue from product sales to date, and we may never 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; further 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:

- our ability to generate revenue from XOLREMDI;
- outcomes and timing of regulatory reviews, approvals and other actions;
- our ability to manufacture any approved products on commercially reasonable terms;

- our ability to maintain an effective sales and marketing organization or suitable third-party alternatives for any approved products;
- the scope, number, progress, duration, endpoints, cost, results and timing of clinical trials and nonclinical studies of our current or potential future product candidates, including in particular the scope, progress, duration, endpoints, cost, results and timing for completion of our Phase 2 and Phase 3 clinical trial of mavorixafor for the treatment of chronic neutropenic disorders;
- our ability to raise sufficient funds to support the development and potential commercialization of our product candidates;
- our ability to market our approved product and obtain marketing approval for our product candidates;
- our ability to 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 operating plan and liquidity position 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.

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 to continue to incur research and development expenses as we continue to advance the clinical development of our product candidates and prepare for the launch and commercialization of any product candidates for which we receive regulatory approval. We expect to incur significant commercialization expenses related to product sales, marketing, distribution and manufacturing. Furthermore, we expect to incur additional costs associated with operating as a public company.

As of June 30, 2024, we have cash and cash equivalents of $147.2 million and marketable securities of $21.5 million. Although we believe we have liquidity to continued operations for at least the next 12 months from the issuance of these consolidated financial statements, we will require additional capital to sustain our operations, and to carry out our business plans thereafter, 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 at a level greater than 20% of our outstanding borrowings under the Hercules Loan Agreement and

subject to certain operational covenants. Based on our current cash flow projections, excluding additional sources of external financing, we anticipate that we will be able to maintain the minimum cash required to satisfy this covenant for at least the next 12 month period following the issuance of these condensed consolidated financial statement. See also the risk factor titled "*Our term loan contains restrictions that limit our flexibility in operating our business*" below.

To finance our future operations beyond the one-year period following the issuance of these condensed consolidated financial statements, we will need to raise additional capital, which cannot be assured. 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. If we default on such indebtedness, with Hercules or a future lender, we could be required to pledge additional assets, or the lenders could enforce remedies on the current collateral.

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

Changes in estimates regarding fair value of intangible assets may result in an adverse impact on our results of operations.
We test our goodwill for impairment annually or more frequently if changes in circumstances or the occurrence of events suggest impairment exists. Any significant change in market conditions, including a sustained decline in our stock price, that indicate a reduction in carrying value may give rise to 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 there were no triggering events during the three months ended June 30, 2024 that necessitated an interim impairment test of goodwill, 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 have not generated significant revenues from product sales since inception and may never become profitable. We may never be able to generate meaningful revenues from sales of XOLREMDI at levels or on timing necessary to support our investment and goals.***
To date, we have not generated significant revenues from product sales and cannot predict whether or when we will be able to generate meaningful revenues from sales of 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 commercialize XOLREMDI and to 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;
- further develop a commercial organization capable of sales, marketing and distribution for the products we intend to sell ourselves in the markets in which we have retained commercialization rights;
- find suitable collaborators to help us market, sell and distribute our approved 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 meaningful revenues from the sale of our product, we may not become profitable and may need to obtain additional funding to continue operations. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce our operations. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of the company and could impair our ability to raise capital, maintain our discovery and preclinical development efforts, expand our business or continue our operations and may require us to raise additional capital that may dilute your ownership interest. A decline in our value could also cause you to lose all or part of your investment.

If the commercial opportunity for mavorixafor in 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.

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 $13.0 million, contingent upon our achievement of certain late-stage regulatory and sales milestones with respect to licensed products.
- our obligation to pay Genzyme tiered royalties based on net sales of licensed products that we commercialize under the agreement.
- our obligation to pay Genzyme a certain percentage of cash payments received by us or our affiliates in consideration for the grant of a sublicense under the license granted to us by Genzyme.

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

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

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

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

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

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

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

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain any of our license agreements on acceptable terms, we may be unable to successfully develop and commercialize the affected product 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 and Phase 3 clinical trials 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.

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, or impose ongoing requirements for potentially costly post-approval studies or post-market surveillance. Progress reports are required at quarterly intervals, every six months and at annual intervals depending upon the country, and more frequently if serious adverse events occur.

Our approved product 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 will be limited to those indications and populations that are specifically approved by the FDA or such other regulatory agencies, and if we are found to have promoted such off-label uses, we may become subject to significant liability. For example, the federal government has levied large civil and criminal fines against companies for alleged improper promotion and in some instances has also required companies to enter into corporate integrity agreements or imposed permanent injunctions under which specified promotional conduct is changed or curtailed. If we cannot successfully manage the promotion of our products, we could become subject to significant liability, which would materially adversely affect our business and financial condition.

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

Our approved product may not gain market acceptance among hospitals, physicians, health care payors, patients and the medical community. Market acceptance of any of our approved product or product candidates for which we receive approval in the future 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 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 though we have obtained approval for one of 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. Moreover, the U.S. Supreme Court's June 2024 decision to overturn established case law giving deference to regulatory agencies' interpretations of ambiguous statutory language has introduced uncertainty regarding the extent to which the FDA's regulations, policies and decisions may become subject to increasing legal challenges, delays, and/or changes.

See the sections of this Annual Report on Form 10-K for the fiscal year ended December 31, 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 limited experience manufacturing our product or product candidates on a large clinical or commercial scale and have no manufacturing facility. We are currently dependent on a single third party manufacturer for the manufacture of the active pharmaceutical ingredient ("API") for mavorixafor, and a single manufacturer of mavorixafor finished drug product

capsules. If we experience problems with these third parties, the manufacturing of mavorixafor could be delayed, which could harm our results of operations.
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 sale of our product.

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

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

We are exposed to the risk that our employees, principal investigators, CROs, CMOs and consultants may engage in fraudulent conduct or other illegal activity. Misconduct by these parties could include intentional failures to comply with FDA regulations or similar regulations of comparable foreign regulatory authorities, to provide accurate information to the FDA or comparable foreign regulatory authorities, to comply with manufacturing standards we have established, to comply with federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by comparable foreign regulatory authorities, to report financial information or data accurately or to disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Employee or third party misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter employee misconduct and the precautions we take to detect and prevent this activity, such as employee training, may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of significant fines or other sanctions.

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

Our drug development programs and the potential commercialization of our product candidates will require substantial additional cash to fund expenses. For some of our product candidates, we may decide to collaborate with pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge and industry and market conditions generally. The collaborator may also consider alternative product candidates for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidates.

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

We have, and may selectively seek in the future, third-party collaborators for the development and commercialization of our product candidates. Our likely collaborators for any collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. If we enter into any such arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates. Our ability to generate revenue from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements.

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 June 30, 2024, we had 127 full-time employees. As our development and commercialization plans and strategies develop, we may 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 ("Section 382") contains rules that limit the ability of a company that undergoes an ownership change to utilize its net operating losses ("NOLs") and tax credits existing as of the date of such ownership change. Under the rules, such an ownership change is generally any change in ownership of more than 50% of a company's stock within a rolling three-year period. The rules generally operate by focusing on changes in ownership among stockholders considered by the rules as owning, directly or indirectly, 5% or more of the stock of a company and any change in ownership arising from new issuances of stock by the company. We have 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.
Our Hercules Loan Agreement is secured by a lien on substantially all of our assets, excluding intellectual property. This loan contains various covenants that limit our ability to engage in specified types of transactions. These covenants limit our ability to, among other things:

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

We also have a covenant under our Hercules Loan Agreement that requires that we maintain a minimum level of cash of $20.0 million through January 2025, and thereafter at a level greater than 20% of our outstanding borrowings under the Hercules Loan Agreement and subject to certain operational covenants.

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 to successfully commercialize XOLREMDI;
- 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, 2024. 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 June 30, 2024, the Company's six section 16 officers adopted written plan for the purchase or sale of Company securities intended to satisfy the affirmative defense conditions of Rule 10b5-1(c). These plans were adopted on June 28, 2024, and are subject to a 90-day "cool off" period before any trades may be conducted under such plans.

Name	Titles	Action Taken	Type of Trading	Nature of Trading	Expiration Date	Aggregate Number of Securities
Christophe Arbet-Engels	Chief Medical Officer	Adoption (June 28, 2024)	Rule 10b5-1 trading arrangement	Sale	8/1/2025	(i) 572,917 shares of common stock subject to employee stock options and (ii) Indeterminable (2).
Mark Baldry	Chief Commercial Officer	Adoption (June 28, 2024)	Rule 10b5-1 trading arrangement	Sale	10/1/2025	Indeterminable (1)
Mary DiBiase	Chief Operating Officer	Adoption (June 28, 2024)	Rule 10b5-1 trading arrangement	Sale	10/1/2025	Indeterminable (1)
Adam Mostafa	Chief Financial Officer	Adoption (June 28, 2024)	Rule 10b5-1 trading arrangement	Sale	10/1/2025	Indeterminable (2)
Paula Ragan	President and Chief Executive Officer	Adoption (June 28, 2024)	Rule 10b5-1 trading arrangement	Sale	10/1/2025	(i) 313,794 shares of common stock subject to long, restricted and control stock and (ii) Indeterminable (1)
Arthur Taveras	Chief Scientific Officer	Adoption (June 28, 2024)	Rule 10b5-1 trading arrangement	Sale	10/1/2025	Indeterminable (1)

(1) The number of shares that will be sold in accordance with this trading arrangement is indeterminable at the time of the filing of this Quarterly Report on Form 10-Q for the period ended June 30, 2024. Upon vesting of PRSUs, a sell-to-cover trade may occur or the Company may withhold shares in order to satisfy any applicable tax withholding obligations on behalf of the employee. The number of shares sold in the sell-to-cover will vary based on the market price of the Company's common stock at the time of settlement.

(2) The number of shares that will be sold in accordance with this trading arrangement is indeterminable at the time of the filing of this Quarterly Report on Form 10-Q for the period ended June 30, 2024. Upon vesting of PRSUs, a sell-to-cover trade may occur or the Company may withhold shares in order to satisfy any applicable tax withholding obligations on behalf of the employee. The number of shares sold in the sell-to-cover will vary based on the market price of the Company's common stock at the time of settlement. Any remaining shares held by the seller after the sell-to-cover may be sold pursuant to the terms of this trading arrangement.

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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
10.1*^	Asset Purchase Agreement, dated May 8, 2024				
10.2*#	Non-Employee Director Deferred Compensation Policy				
10.3*	Insider Trading Policy				
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.
^ Portions of this exhibit (indicated by asterisks) have been omitted pursuant to Item 601(b)(10) of Regulation S-K.
# Indicates management contract or compensatory plan, contract or agreement.

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: August 8, 2024

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

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

Date: August 8, 2024

By: /s/ Adam S. Mostafa

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

EXHIBIT 10.1

CERTAIN CONFIDENTIAL INFORMATION MARKED BY [***] HAS BEEN EXCLUDED FROM THIS EXHIBIT BECAUSE IT IS BOTH NOT MATERIAL AND IS THE TYPE THAT THE REGISTRANT TREATS AS PRIVATE OR CONFIDENTIAL

ASSET PURCHASE AGREEMENT

BY AND BETWEEN

[***]

AND

X4 PHARMACEUTICALS, INC.

MAY 8, 2024

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List of Exhibits

ASSET PURCHASE AGREEMENT

This ASSET PURCHASE AGREEMENT (this "***Agreement***") is made and entered into as of May 8, 2024 (the "***Effective Date***") by and between ***[***]*** ("***Buyer***") and **X4 PHARMACEUTICALS, INC.**, a Delaware corporation ("***Seller***"). Buyer and Seller may hereinafter be referred to individually as a *"**Party**"* and collectively as the "***Parties***."

RECITALS

WHEREAS, Seller is the holder of all right, title and interest in and to the Priority Review Voucher (as defined below);

WHEREAS, Seller and Buyer each (a) desire that Buyer purchase from Seller, and Seller sell, transfer and assign to Buyer, the Purchased Assets (as defined below), all on the terms set forth herein (such transaction, the "***Asset Purchase***") and (b) in furtherance thereof, have adopted and approved this Agreement and, upon the terms and subject to the conditions set forth in this Agreement, have adopted and approved the Asset Purchase as contemplated by this Agreement in accordance with all applicable Legal Requirements (as defined below); and

WHEREAS, Seller and Buyer desire to make certain representations, warranties, covenants, and other agreements as set forth herein in connection with the Asset Purchase contemplated by this Agreement.

NOW, THEREFORE, in consideration of the foregoing and their mutual undertakings hereinafter set forth, and for good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged, the Parties, intending to be legally bound, agree as follows:

ARTICLE 1
DEFINITIONS

1.1 Certain Definitions. As used in this Agreement, the following capitalized terms shall have the meanings indicated below:

(a) "***Action***" means any claim, audit, examination, action, cause of action or suit (whether in contract or tort or otherwise), litigation (whether at law or in equity, whether civil or criminal), assessment, arbitration, mediation, investigation, hearing, charge, complaint, demand, notice, or proceeding.

(b) "***Affiliate***" means, with respect to any Party, any Person that, directly or indirectly through one or more intermediaries, controls, is controlled by, or is under common control with, such Party, for so long as such control exists, whether such Person is or becomes an Affiliate on or after the Effective Date. A Person shall be deemed to "control" another Person if it: (i) owns, directly or indirectly, beneficially or legally, at least fifty percent (50%) of the outstanding capital stock, voting securities, or other ownership interest (or such lesser percentage which is the maximum allowed to be owned by such Person in a particular jurisdiction) of such other Person (or, with respect to a limited partnership or other similar entity, its general partner or

controlling entity) or (ii) has the power, whether pursuant to Contract, ownership of securities or otherwise, to direct the management and policies of such other Person.

(c) "***Agreement***" has the meaning set forth in the Preamble.

(d) "***Approval Letter***" means the NDA 218709 approval letter, dated April 26, 2024, from the FDA to Seller, reflecting the FDA Approval and the grant of the Priority Review Voucher attached hereto as Exhibit A.

(e) "***Asset Purchase***" has the meaning set forth in the Recitals.

(f) "***Business Day***" means a day (i) other than Saturday or Sunday and (ii) on which commercial banks are open for business in New York, New York, United States.

(g) "***Buyer***" has the meaning set forth in the Preamble.

(h) "***Confidential Information***" means ***[***]***.

(i) "***Confidentiality Agreement***" means that certain confidentiality agreement by and between ***[***]*** and Seller, dated ***[***]***.

(j) "***Consent***" means any and all filings, authorizations, consents, approvals, notices, permits, orders, registrations, or declarations.

(k) "***Contract***" means any written or oral legally binding contract, agreement, instrument, commitment, or undertaking (including leases, licenses, mortgages, notes, guarantees, sublicenses, subcontracts, and purchase orders).

(l) "***Effective Date***" has the meaning set forth in the Preamble.

(m) "***Encumbrance***" means any lien, pledge, charge, mortgage, owner's mortgage, easement, encroachment, imperfection of title, title exception, title defect, right of possession, right of negotiation or refusal, leasehold interest, security interest, encumbrance, adverse claim, interference, or other restriction on transfer, ownership, or use.

(n) "***FDA***" means the U.S. Food and Drug Administration.

(o) "***FDA Approval***" means the marketing authorization for XOLREMDI™ (mavorixafor) capsules issued by the FDA to Seller effective on April 26, 2024, relating to NDA 218709, which was submitted under Section 505(b) of the FFDCA.

(p) "***FFDCA***" means the United States Federal Food, Drug, and Cosmetic Act, 21 U.S.C. § 301 *et seq.*, as amended from time to time, together with any rules, regulations, and requirements promulgated thereunder (including all additions, supplements, extensions, and modifications thereto).

(q) "***Fundamental Breach Event***" has the meaning set forth in Section 6.7.

(r) "***Fundamental Representations***" means the representations and warranties contained in Section 3.1 (Organization; Standing and Power), Section 3.2 (Due Authority), Section 3.3 (No Contravention), Section 3.5 (Title to Purchased Assets), Section 3.6 (Compliance with Legal Requirements), Section 3.9 (Revocation; Use of Purchased Assets), Section 3.10 (Marketed Product), Section 3.11 (Brokers) and Section 3.12 (Taxes).

(s) "***Governmental Entity***" means any supranational, national, state, municipal, local or foreign government, any court, tribunal, arbitrator, administrative agency, commission, or other governmental official, authority, or instrumentality, in each case, whether domestic or foreign, any stock exchange or similar self-regulatory organization, or any quasi-governmental, private body or arbitral body exercising any executive, legislative, judicial, quasi-judicial, regulatory, taxing, importing, administrative, or other governmental or quasi-governmental authority.

(t) "***Indemnified Party***" means any of the Buyer Indemnified Parties or Seller Indemnified Parties, as applicable.

(u) "***Indemnifying Party***" means any Person against whom a claim for indemnification is being asserted under any provision of ARTICLE 6.

(v) "***Indirect Taxes***" has the meaning set forth in Section 2.8.

(w) "***Judgment***" means any orders, writs, injunctions, awards, judgments, settlements, stipulations, determinations, and decrees entered by or with any Governmental Entity.

(x) "***Knowledge***" means, with respect to Seller, the actual knowledge of the facts and information of any director, officer, or member of senior management, of Seller who would reasonably be expected to know such information due to the nature of such person's positions or duties, after performing a reasonable inquiry with respect to such facts and information.

(y) "***Law***" means any federal, state, foreign, local, municipal, or other law, statute, constitution, principle of common law, resolution, ordinance, code, edict, decree, rule, regulation, ruling or requirement issued, enacted, adopted, promulgated, implemented, or otherwise put into effect by or under the authority of any Governmental Entity.

(z) "***Legal Requirement***" means any federal, state, foreign, local, municipal or other law, statute, constitution, principle of common law, resolution, ordinance, code, rule, regulation, ruling or requirement issued, enacted, adopted, promulgated, implemented or otherwise put into effect by or under the authority of any Governmental Entity and any Orders applicable to a Party or to any of its assets, properties or businesses. Legal Requirements shall include, with respect to Seller or its Affiliates, any responsibilities, requirements, parameters and conditions relating to the Priority Review Voucher set forth in (i) the Approval Letter, (ii) any other correspondence received by Seller or its Affiliates from the FDA regarding the Priority Review Voucher, (iii) Section 529 of the FFDCA (21 U.S.C. § 360ff), or (iv) in the FDA's Draft Guidance, "Rare Pediatric Disease Priority Review Vouchers —Guidance for Industry" (July 2019).

(aa) "***Liabilities***" means ***[****]***.

(bb) "***Losses***" means ***[***]***.

(cc) "***Market,***" "***Marketed***" or "***Marketing***" means to market a drug as described in Section 529(e)(1) of the FFDCA.

(dd) "***Marketing Period***" has the meaning set forth in Section 3.10.

(ee) "***Marketing Requirement***" has the meaning set forth in Section 5.6.

(ff) "***Person***" means any natural person, company, corporation, limited liability company, general partnership, limited partnership, trust, proprietorship, joint venture, business organization, or Governmental Entity.

(gg) "***PHSA***" means the United States Public Health Service Act, 42 U.S.C. § 201 et seq., as amended from time to time, together with any rules, regulations, and requirements promulgated thereunder (including all additions, supplements, extensions, and modifications thereto).

(hh) "***Priority Review***" has the meaning given in Section 529(a)(1) of the FFDCA.

(ii) "***Priority Review Fee***" has the meaning given is Section 529(c) of the FFDCA.

(jj) "***Priority Review Voucher***" means the priority review voucher issued by the Secretary of the Department of Health and Human Services pursuant to Section 529(b)(1) of the FFDCA to Seller and assigned tracking number PRV NDA 218709 that entitles the holder of such voucher to Priority Review of a single human drug application submitted under Section 505(b)(1) of the FFDCA or section 351(a) of the PHSA.

(kk) "***Purchased Assets***" means (i) the Priority Review Voucher and (ii) any and all rights, benefits and entitlements with respect thereto afforded to the holder of such Priority Review Voucher.

(ll) "***Rare Pediatric Disease***" means rare pediatric disease as defined in Section 529(a)(3) of the FFDCA.

(mm) "***Representative***" means, with respect to a particular Person, any director, officer, manager, employee, agent, consultant, advisor, accountant, financial advisor, legal counsel, or other representative of that Person.

(nn) "***SEC***" has the meeting set forth in Section 5.3.

(oo) "***Tax***" or "***Taxes***" means ***[***]***.

(pp) "***Third Party***" means any Person other than a Party and such Party's Affiliates.

(qq) "***Transfer Taxes***" has the meaning set forth in Section 2.7.

(rr) "***Unstated Indirect Taxes***" has the meaning set forth in Section 2.8.

(ss) "***U.S.***" means United States of America.

Other capitalized terms defined elsewhere in this Agreement and not defined in this Section 1.1 shall have the meanings assigned to such terms in this Agreement.

ARTICLE 2
PURCHASE AND SALE

2.1 Purchase and Sale of Purchased Assets.

(a) Upon the terms and subject to the conditions of this Agreement, Buyer hereby purchases from Seller and Seller, on behalf of itself and its Affiliates, hereby irrevocably sells, transfers, conveys, assigns, and delivers to Buyer all of Seller's and its Affiliates' rights, title, and interests in and to the Purchased Assets free and clear of all Encumbrances. Seller shall perform all actions necessary to facilitate the transfer of the Purchased Assets to Buyer.

(b) Notwithstanding anything in this Agreement to the contrary, neither Buyer nor any of its Affiliates shall assume, nor shall Buyer or any of its Affiliates be liable for, or otherwise be obligated to pay, perform, or discharge, any Liabilities of Seller or its Affiliates, including any Liabilities arising from or related to Seller's ownership prior to the Effective Date of any rights with respect to the Purchased Assets (other than obligations that are imposed generally by applicable Legal Requirements solely on the holder of the Priority Review Voucher in respect of its use or transfer following the sale thereof pursuant to this Agreement, including the Priority Review Fee and any other user fees required to be paid to redeem the Priority Review Voucher) (such Liabilities, the "***Excluded Liabilities***").

2.2 Purchase Price. The total consideration to be paid by Buyer for all of the Purchased Assets shall be ONE HUNDRED AND FIVE MILLION U.S. DOLLARS (U.S. $105,000,000.00) (the "***Purchase Price***"). Buyer shall pay the Purchase Price to Seller by 5:00 pm ET on the Effective Date in United States dollars by wire transfer of immediately available funds to the following bank account of Seller:

Bank Name: ***[***]***.
Bank Address: ***[***]***
ABA: ***[***]***Account Number: ***[***]***

2.3 Title Passage; Delivery of Purchased Assets.

(a) *Title Passage*. Upon the execution of this Agreement, all of the rights, title, and interests in and to the Purchased Assets shall pass to Buyer free and clear of all Encumbrances.

(b) *Method of Delivery of Assets*. Within three (3) Business Days following the Effective Date, Seller shall submit, or cause to be submitted, to the FDA the separate notifications referred to in Section 2.4(b) and Section 2.5(c), respectively, as a submission to NDA 218709

through the FDA's Electronic Submissions Gateway under the cover letter in the form attached as Exhibit 2.3(b). Seller shall provide to Buyer, within two (2) Business Days following their submission to the FDA, confirmation from the FDA of successful submission and a complete copy of such submission.

(c) *Filings; Notifications*. Buyer and Seller agree to cooperate and assist each other with respect to all filings and notifications to the FDA related to the transfer and assignment of the Purchased Assets.

2.4 Deliveries by Seller. Upon the execution of this Agreement, Seller shall deliver, or cause to be delivered, to Buyer the following:

(a) a duly executed counterpart of the Bill of Sale substantially in the form attached hereto as Exhibit 2.4(a);

(b) a copy of the notification of the purchase and sale of the Priority Review Voucher pursuant to this Agreement to be submitted to the FDA by, or on behalf of, Seller pursuant to Section 2.3(b), which notification shall be in the form of Exhibit 2.4(b) or such other form as the FDA may require as of the Effective Date; and

(c) a properly completed, validly executed, true and correct Internal Revenue Service Form W-9 certifying that Seller is not subject to backup withholding for United States federal income tax purposes.

2.5 Deliveries by Buyer. Upon the execution of this Agreement, Buyer shall deliver, or cause to be delivered, to Seller the following:

(a) payment of the Purchase Price in accordance with Section 2.2;

(b) a duly executed counterpart of the Bill of Sale substantially in the form attached hereto as Exhibit 2.4(a); and

(c) a copy of the notification of the purchase and sale of the Priority Review Voucher pursuant to this Agreement to be submitted to the FDA by, or on behalf of, Seller pursuant to Section 2.3(b), which notification shall be in the form of Exhibit 2.5(c) or such other form as the FDA may require as of the Effective Date.

2.6 Withholding. Buyer shall be entitled to deduct and withhold from any payments contemplated by this Agreement to be paid by Buyer to any Person the amounts Buyer is required to deduct and withhold pursuant to applicable Law. Amounts so withheld and paid over to the appropriate Governmental Entity shall be treated for all purposes of this Agreement as having been paid to the applicable Person in respect of which such deduction and withholding was made.

2.7 Transfer Taxes. Notwithstanding any other provision in this Agreement to the contrary, any transfer Taxes, documentary charges, recording fees, and similar Taxes, charges, or fees (including any penalties, interest and additions thereto) that may become payable by either Party or its Affiliates in connection with the sale of the Purchased Assets to Buyer (collectively, "***Transfer Taxes***") shall be economically borne by Seller. Any Transfer Tax shall be paid to the

applicable Governmental Entity by the Party that is primarily liable for payment of such Tax under applicable Law. Seller shall promptly reimburse Buyer for any Transfer Taxes paid by Buyer in accordance with this Section 2.7. Any such payment by Seller to Buyer pursuant to this Section 2.7 shall be treated as a reduction of and adjustment to the Purchase Price for all Tax purposes, unless otherwise required by applicable Law. Buyer and Seller shall reasonably cooperate in preparing and timely filing any Tax returns required to be filed in respect of any Transfer Taxes. Buyer shall use commercially reasonable efforts to assist Seller in mitigating, reducing or eliminating any Transfer Tax. For the avoidance of doubt, Transfer Taxes exclude any Indirect Taxes.

2.8 Indirect Taxes. All amounts mentioned in this Agreement are exclusive of any value added, goods and services, sales, use and similar Taxes ("***Indirect Taxes***"). Seller shall issue all invoices in full compliance with the Indirect Tax laws and regulations applicable at Seller's place of business or in any other jurisdiction where any of the transactions contemplated by this Agreement are executed, or deemed to be executed according to local Law, with respect to any Indirect Taxes. If any Indirect Taxes are due based on local Law, Seller will be allowed to add the amount of Indirect Taxes to the amounts mentioned in this Agreement and invoice the net amount plus the applicable Indirect Taxes; *provided* that, if any applicable Indirect Taxes are not separately stated on any invoice (the "***Unstated Indirect Taxes***"), then Seller shall be responsible for any interest, penalties, additions to or increases of the applicable Indirect Taxes due hereunder that are attributable to such Unstated Indirect Taxes. The Parties shall issue invoices for all amounts payable under this Agreement consistent with all Indirect Tax requirements and irrespective of whether the sums may be netted for settlement purposes. If Seller is required to pay any Indirect Taxes, Seller shall provide Buyer with evidence that such Indirect Taxes have been paid, and Buyer shall reimburse Seller for such Indirect Taxes (other than any interest, penalties and addition to or increases of the applicable Unstated Indirect Taxes that are borne by Seller pursuant to this Section 2.8). Buyer and Seller shall reasonably cooperate in preparing and timely filing any tax returns required to be filed in respect of any Indirect Taxes. Seller shall use commercially reasonable efforts to assist Buyer in mitigating, reducing or eliminating any Indirect Taxes, including but not limited to cooperating with Buyer in providing any information or documentation that may be necessary to obtain such mitigation, reduction or elimination.

2.9 Apportioned Obligations. All *ad valorem* obligations levied with respect to the Purchased Assets for a taxable period that includes (but does not end on) the Effective Date shall be apportioned between Seller and Buyer on a *per diem* basis. Seller shall be liable for the proportionate amount of such *ad valorem* obligations that is attributable to the portion of such taxable period ending at the end of the Effective Date, and Buyer shall be liable for the remainder of such obligations.

2.10 Broker Fees. Notwithstanding any other provision in this Agreement to the contrary, Seller shall bear and pay any and all fees and expenses that may become payable by either Party or its Affiliates in connection with any arrangement made by Seller or its Affiliates with any broker, finder or investment banker in connection with the purchase and sale of the Purchased Assets hereunder or any of the other transactions contemplated by this Agreement.

ARTICLE 3
REPRESENTATIONS AND WARRANTIES OF SELLER

Seller hereby represents and warrants to Buyer, as of the Effective Date (or in the case of representations and warranties that are made as of a specified date, as of such specified date) as follows:

3.1 Organization, Standing, and Power. Seller is a corporation duly organized, validly existing, and in good standing under the laws of Delaware. Seller has the requisite corporate power and authority to own, operate, and lease its properties and to carry on its business as presently conducted and is duly qualified or licensed to do business and is in good standing in each jurisdiction where the character of its properties owned or leased or the nature of its activities make such qualification or licensing necessary, except where the failure to be so qualified or licensed would not, individually or in the aggregate, reasonably be expected to adversely affect any of the Purchased Assets, Seller's ability to consummate the transactions contemplated by this Agreement, or Buyer's ownership and rights with respect to any of the Purchased Assets after the Effective Date. Seller is not in violation of its organizational documents, as amended to date.

3.2 Due Authority. Seller has all requisite corporate power and authority to execute and deliver, perform its obligations under, and consummate the transactions contemplated by this Agreement. The execution, delivery, and performance of this Agreement, and the consummation of the Asset Purchase, have been duly and validly authorized by all necessary corporate action on the part of Seller. This Agreement has been duly executed and delivered by Seller. This Agreement, upon due execution and delivery by the Parties, will constitute a valid and binding obligation of Seller enforceable against Seller in accordance with its terms, subject only to the effect, if any, of (a) applicable bankruptcy and other similar Laws affecting the rights of creditors generally and (b) rules of Law governing specific performance, injunctive relief, and other equitable remedies (whether considered in an action at Law or in equity).

3.3 No Contravention. The execution and delivery by Seller of this Agreement does not, and the consummation of the transactions contemplated hereby, including the transfer of title to, ownership in, and possession of the Purchased Assets, will not (a) result in the creation of any Encumbrance on the Purchased Assets or (b) conflict with, or result in any violation of or default under (with or without notice or lapse of time, or both), or give rise to a right of termination, revocation, suspension, cancellation, or acceleration of any obligation or loss of any benefit under, or (except for the letters referenced in Section 2.3(b)) require any consent, approval, or waiver from any Person pursuant to, (i) any provision of the organizational or governing documents of Seller, in each case as amended to date, (ii) the Priority Review Voucher, the Approval Letter or any Contract to which Seller or any Affiliate of Seller is a party or bound which involves or affects in any way any of the Purchased Assets, or (iii) any Legal Requirements applicable to Seller or any Affiliate of Seller or any of the Purchased Assets.

3.4 No Consents. Except for the letters referenced in Section 2.3(b), no Consent of a Governmental Entity or any other Person is necessary or required in connection with the execution, delivery and performance by Seller of this Agreement, and the consummation by Seller or its Affiliates of the transactions contemplated hereby.

3.5 Title to Purchased Assets. Seller is the sole and exclusive owner of all rights, title, and interests in and to the Purchased Assets and owns good and transferable title to the Purchased Assets free and clear of any Encumbrances. Seller has performed all actions necessary to perfect its ownership of, and its ability to transfer, the Purchased Assets. Neither Seller nor any of its Affiliates has sold, transferred, conveyed, assigned, or delivered any Purchased Assets, or offered to do so, to any Person, and Seller has the full and sole right to sell, transfer, convey, assign, and deliver the Purchased Assets to Buyer free and clear of all Encumbrances.

3.6 Compliance with Legal Requirements. Seller and its Affiliates are, and at all times have been, in compliance with all Legal Requirements that are or were applicable to (a) Seller's and its Affiliates' conduct, acts, or omissions with respect to any of the Purchased Assets or (b) any of the Purchased Assets. None of Seller or any of its Affiliates has received any notice or other communication from any Person regarding any actual or alleged, possible, or potential violation of, or failure to comply with, any such Legal Requirement. Since the **/***/**period prior to the Effective Date and as it relates to the FDA Approval, the Approval Letter, the Priority Review Voucher or the activities giving rise to such FDA Approval, the Approval Letter or the Priority Review Voucher, neither Seller, any Affiliate of Seller, nor to the Knowledge of Seller, any representative of Seller or any Affiliate of Seller, has made an untrue statement of material fact or a fraudulent statement to the FDA or any other Governmental Entity, failed to disclose a material fact or a fraudulent statement to the FDA or any other Governmental Entity or committed an act, made a statement or failed to make a statement that, at the time such disclosure was made, would reasonably be expected to provide a basis for the FDA to revoke the Priority Review Voucher or invoke its policy respecting "Fraud, Untrue Statements of Material Facts, Bribery, and Illegal Gratuities," set forth in 56 Fed. Reg. 46191 (September 10, 1991) or for any other Governmental Entity to invoke any similar policy.

3.7 Legal Proceedings. There is no pending or, to the Seller's Knowledge, threatened Action involving Seller or any of its Affiliates, nor has there been any Action involving Seller or any of its Affiliates, and neither Seller nor any of its Affiliates are a party or subject to the provisions of any Judgment, in each case, (a) that involves or affects (or may involve or affect) the issuance of, continued validity of, ownership of, transfer or license of, title to, or use of any of the Purchased Assets, including any such Action or Judgment that seeks to prohibit or limit in any respect, or place any conditions on, the ownership or use by Buyer or its Affiliates of any of the Purchased Assets, in each case, as a result of the transactions contemplated by this Agreement, or (b) that otherwise challenges or seeks to restrain, prohibit, prevent, enjoin, alter, or delay the consummation of the transactions contemplated by this Agreement.

3.8 Governmental Authorizations. Neither Seller nor any of its Affiliates is required to hold any license, registration, or permit issued by any Governmental Entity to own, use, or transfer the Purchased Assets, other than such licenses, registrations, or permits that have already been obtained.

3.9 Revocation; Use of Purchased Assets. The Priority Review Voucher has been duly granted and issued and has not been revoked, and there are no facts or circumstances that would reasonably be expected to result in the revocation of the Priority Review Voucher by a Governmental Entity, give rise to a right of the FDA to revoke the Priority Review Voucher, or result in the redemption or transfer of the Priority Review Voucher (other than pursuant to the

transactions contemplated by this Agreement), or that would reasonably be expected to preclude or interfere with the sale and transfer of the Purchased Assets to Buyer or Buyer's use of the Purchased Assets following the Effective Date to obtain Priority Review or any other benefits associated with the Purchased Assets. Except for this Agreement, there is no Contract to which Seller or any Affiliate of Seller is a party that involves or affects the ownership of, licensing of, title to, sale or other transfer of, or use of any of the Purchased Assets. There is no term or condition imposed by the FDA as of the date hereof on the Priority Review Voucher that is not set forth in the Approval Letter or provided for under applicable Law. Seller has provided to Buyer true and complete copies of the Approval Letter and all other material written correspondence between Seller or any of its Affiliates and the FDA regarding the Priority Review Voucher, in each case, (a) with such redactions to any portion of the Approval Letter and such other material written correspondence that is not relevant to the Priority Review Voucher and (b) including any and all corrections with respect thereto. Neither Seller nor any of its Affiliates has notified the FDA, or permitted any Third Party to notify the FDA, of intent to use the Priority Review Voucher.

3.10 Marketed Product. Seller and its Affiliates have commenced, will commence, or Seller shall cause its Affiliates to commence, Marketing in the United States of the Rare Pediatric Disease product for which the Priority Review Voucher was awarded ***[***]*** period beginning on the date of the FDA Approval of such Rare Pediatric Disease product (such period, the "***Marketing Period***").

3.11 Brokers. No Broker, finder, or investment banker is entitled to any brokerage or finder's fee in connection with the purchase and sale of the Purchased Assets hereunder or any of the other transactions contemplated by this Agreement based upon arrangements made by or on behalf of Seller or its Affiliates.

3.12 Taxes. Seller and its Affiliates have timely paid any amount of Tax required to be paid on or prior to the date hereof, if a failure to pay such Tax would result in a lien on any of the Purchased Assets. There are no liens on account of Taxes on the Purchased Assets and no material audits, controversies or claims by a Governmental Entity pending or threatened against Seller with respect to Taxes relating to the Purchased Assets.

ARTICLE 4
REPRESENTATIONS AND WARRANTIES OF BUYER

Buyer hereby represents and warrants to Seller as of the Effective Date as follows:

4.1 Organization, Standing, and Power. Buyer is a limited liability company duly organized, validly existing, and in good standing under the laws of Delaware. Buyer has the requisite power and authority to own, operate, and lease its properties and to carry on its business as presently conducted and is duly qualified or licensed to do business and is in good standing in each jurisdiction where the character of its properties owned or leased or the nature of its activities make such qualification or licensing necessary, except where the failure to be so qualified or licensed would not, individually or in the aggregate, reasonably be expected to adversely affect Buyer's ability to consummate the transactions contemplated by this Agreement.

4.2 Authority. Buyer has all requisite power and authority to execute and deliver, perform its obligations under, and consummate the transactions contemplated by, this Agreement. The execution, delivery, and performance of, and the consummation of the transactions contemplated by, this Agreement have been duly and validly approved and authorized by all necessary action on the part of Buyer. This Agreement has been duly executed and delivered by Buyer. This Agreement, upon due execution and delivery by the Parties, will constitute a valid and binding obligation of Buyer, enforceable against Buyer in accordance with its terms, subject only to the effect, if any, of (a) applicable bankruptcy and other similar Laws affecting the rights of creditors generally and (b) rules of Law governing specific performance, injunctive relief and other equitable remedies (whether considered in an action at Law or in equity).

4.3 No Contravention. The execution and delivery by Buyer of this Agreement does not, and the consummation of the transactions contemplated hereby (including the transfer of title to, ownership in, and possession of the Purchased Assets) will not, conflict with, or result in any violation of or default under (with or without notice or lapse of time, or both), or give rise to a right of termination, cancellation, or acceleration of any obligation or loss of any benefit under, (except for the letters referenced in Section 2.3(b)) or require any consent, approval, or waiver from any Person pursuant to, (a) any provision of the organizational or governing documents of Buyer, in each case as amended to date, (b) any Contract to which Buyer or any Affiliate of Buyer is a party or bound by or by which it or its assets or properties are bound or under which Buyer or any Affiliate of Buyer has material rights or benefits or is bound which involves in any way the transactions contemplated in this Agreement, or (c) any Legal Requirements applicable to Buyer.

4.4 No Consents. Except for the letters referenced in Section 2.3(b), no Consent of any Governmental Entity or any other Person is necessary or required in connection with the execution, delivery and performance by Buyer of this Agreement or the consummation by Buyer of the transactions contemplated hereby.

4.5 Brokers. No broker, finder, or investment banker is entitled to any brokerage or finder's fee in connection with the purchase and sale of the Purchased Assets hereunder or any of the other transactions contemplated by this Agreement based upon arrangements made by or on behalf of Buyer or its Affiliates.

4.6 Financing. Buyer has sufficient funds to permit the Buyer to consummate the transactions contemplated by this Agreement. Notwithstanding anything to the contrary contained herein, the Parties acknowledge and agree that it shall not be a condition to the obligations of Buyer to consummate the transactions contemplated hereby that Buyer have sufficient funds for payment of the Purchase Price.

4.7 Notice of Transfer. Buyer acknowledges that it is responsible for notifying the FDA of the transfer of the Purchased Assets and the delivery to the FDA of the letters referenced in Section 2.3(b), in accordance with the requirements of Section 529(b)(2)(B) of the FFDCA and as further described in the FDA's Draft Guidance, "Rare Pediatric Disease Priority Review Vouchers —Guidance for Industry" (July 2019).

4.8 Non-Reliance. Neither Seller nor any of its Affiliates nor any of its Representatives makes, or has made any representation or warranty, oral or written, express or implied, as to the

accuracy or completeness of any information concerning the Purchased Assets contained herein or made available in connection with Buyer's investigation of the foregoing, except as expressly set forth in this Agreement, and Seller, its Affiliates and their Representatives expressly disclaim any and all liability that may be based on such information or errors therein or omissions therefrom. Buyer has not relied and is not relying on any statement, representation or warranty, oral or written, express or implied (including any representation or warranty as to the merchantability or fitness for a particular purpose), made by Seller, any of its Affiliates or any of their Representatives, except as expressly set forth in Article III.

ARTICLE 5
COVENANTS

5.1 Expenses. Whether or not the purchase and sale of the Purchased Assets and the other transactions contemplated by this Agreement are consummated, and except as otherwise set forth in this Agreement, each of the Parties shall bear its own fees and expenses incurred or owed in connection with the purchase and sale of the Purchased Assets, this Agreement, and the transactions contemplated hereby.

5.2 Further Assurances. The Parties shall cooperate reasonably with each other in connection with any steps required to be taken as part of their respective obligations under this Agreement, including without limitation any notifications or filings required to be made to the FDA in connection with the transfer of the Purchased Assets, and shall, at no expense to the other Party, (a) furnish upon request to each other such further information, (b) execute and deliver to each other such other documents, and (c) do such other acts and things, all as the other Party may reasonably request for the purpose of carrying out the intent of this Agreement and the transactions contemplated by this Agreement, including the use of the Purchased Assets to obtain Priority Review. The Parties agree that the user fees to be paid in connection with the use of the Priority Review Voucher by Buyer or any subsequent transferee of the Priority Review Voucher, and all other user fees under the FFDCA applicable to the human drug application for which the Priority Review Voucher is redeemed, shall be borne exclusively by Buyer or any such subsequent transferee of the Priority Review Voucher. In any such event, Seller shall have no liability or obligation for any such fees.

5.3 Public Announcements. Notwithstanding anything in this Agreement to the contrary, except as may be required by applicable Law or as may be required to comply with the requirements of any applicable stock exchange or any Governmental Entity, including the U.S. Securities and Exchange Commission (the "***SEC***"), neither Party shall (a) disclose the existence or terms of this Agreement (other than disclosures to Representatives on a need-to-know basis and who are bound by confidentiality terms substantially no less stringent than the terms of this Agreement) or (b) issue any press release, publication, or other public announcement relating to this Agreement, the performance of this Agreement, or that otherwise identifies the other Party as a party to this Agreement, in each case, without the prior written consent of the other Party, such consent not to be unreasonably withheld, conditioned or delayed. To the extent practicable, the disclosing Party shall give at least ***[***]***advance notice of any legally required disclosure to the non-disclosing Party, and the non-disclosing Party may provide any comments on the proposed legally required disclosure during the foregoing time period; *provided* that such disclosing Party shall be under no obligation to accept any such comments provided by the non-disclosing Party

but shall consider all such comments in good faith. The Parties acknowledge that Seller may be obligated to file a copy of this Agreement with the SEC. Without limiting the foregoing, Seller shall provide Buyer with a reasonable opportunity to review the proposed filing and, if requested by Buyer, Seller shall request, and use reasonable efforts to obtain, confidential treatment of this Agreement pursuant to applicable rules under the Securities Exchange Act of 1934, as amended, and the Freedom of Information Act and the rules promulgated thereunder to permit the filing of a redacted exhibit; *provided* that Buyer acknowledges that there is no assurance that such redactions will be permitted by the SEC and the SEC may require filing of the Agreement in full.

5.4 Use of Name. Except as expressly provided herein, neither Party shall mention or otherwise use the name, logo, or trademark of the other Party or any of its Affiliates (or any abbreviation or adaptation thereof) in any publication, press release, marketing, and promotional material, or other form of publicity or filing that is publicly available without the prior written approval of such other Party in each instance. The restrictions imposed by this Section 5.4 shall not prohibit either Party from making any disclosure identifying the other Party that, in the opinion of the disclosing Party's counsel, is required by applicable Law or the rules of a stock exchange on which the securities of the disclosing Party are listed; *provided* that such disclosing Party shall submit the proposed disclosure identifying the other Party in writing to the other Party as far in advance as reasonably practicable (and in no event less than ***/***/prior to the anticipated date of disclosure) so as to provide a reasonable opportunity to comment thereon.

5.5 Compliance with Legal Requirements. Seller shall, and shall cause its Affiliates and each of their respective successors in interest and assigns to the Rare Pediatric Disease product for which the Priority Review Voucher was awarded to, comply in all material respects at all times with all Legal Requirements applicable to such Persons (as the sponsor of such Rare Pediatric Disease product and the initial recipient and owner of the Priority Review Voucher, as applicable), in any case relating to the Purchased Assets, and comply with any and all Legal Requirements applicable to such Persons that would impact the validity, maintenance, use, or transfer of the Priority Review Voucher, or that would reasonably be expected to result in the revocation of the Priority Review Voucher if such Legal Requirements were not complied with. Seller shall promptly forward to Buyer any communications or notices it or its Affiliates receive from any Governmental Entity to the extent relating directly or indirectly to or otherwise materially impacting the Purchased Assets; *provided*, that Seller may redact any portion of such written communications or other notices that is not relevant to the Priority Review Voucher.

5.6 Marketing. Seller shall, and shall cause its Affiliates and each of their respective successors in interest and assigns to, within the Marketing Period, Market in the United States the Rare Pediatric Disease product for which the Priority Review Voucher was awarded under applicable Legal Requirements to preclude the FDA from exercising its authority to revoke the Priority Review Voucher pursuant to Section 529(e)(1) of the FFDCA (the "***Marketing Requirement***"). Promptly following any request in writing by Buyer, Seller shall notify Buyer in writing as to whether the Marketing Requirement has been satisfied.

5.7 Confidentiality.

(a) With respect to Confidential Information received by any Party, such Party shall (i) keep such Confidential Information confidential, (ii) not use such Confidential

Information for any reason other than to carry out the intent and purpose of this Agreement, and (iii) not disclose such Confidential Information to any Person, except in each case, as otherwise expressly permitted by this Agreement or with the prior written consent of the disclosing Party.

(b) A Party may disclose Confidential Information only to its Representatives on a need-to-know basis and shall (i) enforce the terms of this Section 5.8 as to its Representatives, (ii) take such action to the extent necessary to cause its Representatives to comply with the terms and conditions of this Section 5.8 and (iii) be responsible and liable for any breach of this Section 5.8 by it or its Representatives.

(c) If a Party becomes compelled by a court or is requested by a Governmental Entity to make any disclosure that is prohibited or otherwise constrained by this Section 5.8, such Party shall provide the disclosing Party with prompt written notice of such compulsion or request so that it may seek an appropriate protective order or other appropriate remedy or waive compliance with the provisions of this Section 5.8. In the absence of a protective order or other remedy, the Party subject to the requirement to disclose may disclose that portion (and only that portion) of the Confidential Information that, based upon advice of its counsel, it is legally compelled to disclose or that has been requested by such Governmental Entity; *provided, however*, that such Party shall use reasonable efforts to obtain reliable assurance that confidential treatment will be accorded by any Person to whom any Confidential Information is so disclosed. For the avoidance of doubt, this Section 5.8(c) does not apply to those disclosures concerning this Agreement to which Section 5.3 applies.

(d) Nothing herein shall prohibit or otherwise restrict the disclosure of any Confidential Information by or on behalf of Buyer or its Affiliates to the FDA or other Governmental Entity to the extent required by the FDA or such other Governmental Entity to enable the use or transfer of the Priority Review Voucher; provided, that Buyer, its Affiliates and their respective Representatives shall use commercially reasonable efforts to obtain confidential treatment for any such disclosures.

5.8 Disclosure Concerning Use of the Priority Review Voucher. In the event that Buyer or any of its Affiliates uses (or notifies the FDA of its intent to use) the Priority Review Voucher in connection with a human drug application, Buyer or such Affiliate may, in any press release, public announcement or other disclosure relating to its filing (or proposed filing) of the relevant human drug application, disclose that the Priority Review Voucher acquired from Seller has been used (or is intended to be used) in connection with such human drug application.

ARTICLE 6
INDEMNIFICATION AND LIMITATIONS OF LIABILITY

6.1 Indemnification.

(a) Indemnification by Seller. Seller shall indemnify, defend and hold harmless Buyer and its Affiliates and its and their respective directors, officers, employees, partners, members, agents, Representatives, successors, and assigns (each, a "***Buyer Indemnified Party***") for, from and against any and all Losses, whether or not arising from, relating to, or otherwise in connection with a claim of a Third Party (each, a "***Third Party Claim***"), which any Buyer

Indemnified Party may suffer, incur, sustain, or become subject to, to the extent arising from, relating to or otherwise in connection with (i) any breach of or inaccuracy in any representations and warranties of Seller made under this Agreement or any certificate or document delivered hereunder; (ii) any breach of or failure to perform any covenants or obligations of Seller made under this Agreement or any certificate or document delivered hereunder; (iii) Seller's grossly negligent acts, omissions or misrepresentations or willful misconduct, in each case, in connection with this Agreement; (iv) any and all Excluded Liabilities, and (v) any Fundamental Breach Event.

(b) Indemnification by Buyer. Buyer shall indemnify, defend and hold harmless Seller and its Affiliates, and its and their respective directors, officers, employees, agents, Representatives, successors, and assigns (each, a "***Seller Indemnified Party***") from and against any and all Losses, whether or not arising from, relating to or otherwise in connection with a Third Party Claim, which any Seller Indemnified Party may suffer, incur, sustain, or become subject to, to the extent arising from, relating to or otherwise in connection with (i) any breach of or inaccuracy in any representations and warranties of Buyer made under this Agreement or any certificate or document delivered hereunder; (ii) any breach of or failure to perform any covenants or obligations of Buyer made under this Agreement or any certificate or document delivered hereunder; and (iii) Buyer's grossly negligent acts, omissions or misrepresentations or willful misconduct, in each case, in connection with this Agreement.

6.2 Notice of Loss; Third Party Claims.

(a) A claim for indemnification for any matter not involving a Third Party Claim may be asserted by written notice to the Indemnifying Party. Such notice shall include the facts constituting the basis for such claim for indemnification, the Sections of this Agreement upon which such claim for indemnification is then based and an estimate, if possible, of the amount of Losses suffered or reasonably expected to be suffered by the Indemnified Party; *provided* that the failure to give such notification or any deficiency in such notification will not relieve such Indemnifying Party from any obligation under this ARTICLE 6, except (i) to the extent such failure to give such notification or such deficiency in such notification actually and materially prejudices such Indemnifying Party or (ii) as provided in Section 6.3. If the Indemnifying Party does not notify the Indemnified Party within twenty (20) Business Days following its receipt of such notice that the Indemnifying Party affirmatively accepts liability in the specified amount for the indemnity claimed by the Indemnified Party under Section 6.1(a) or Section 6.1(b), as applicable, such indemnity claim specified by the Indemnified Party in such notice shall be deemed not accepted by the Indemnifying Party, in which case, the Indemnified Party may pursue its right to indemnification with respect to such indemnity claim under this ARTICLE 6 in accordance with the terms hereof.

(b) In the event of any instituted or asserted Third Party Claim against an Indemnified Party, the Indemnified Party shall promptly cause written notice of the assertion of any Third Party Claim of which it has knowledge which is covered by the provisions of Section 6.1(a) or Section 6.1(b), as applicable, to be forwarded to the Indemnifying Party. The failure to give such notification or any deficiency in such notification will not relieve such Indemnifying Party from any obligation under this ARTICLE 6, except (i) to the extent such failure to give such notification or such deficiency in such notification actually and materially prejudices such Indemnifying Party or (ii) as provided in Section 6.3. The Indemnifying Party shall have the right,

at its sole option and expense, to be represented by counsel reasonably acceptable to the Indemnified Party and to defend against, negotiate, settle or otherwise deal with any Third Party Claim which relates to any Losses indemnified by it hereunder, subject to the provisions below; *provided*, *however*, that the Indemnifying Party may not assume control of defense to (or, following such assumption of control in accordance herewith, may not continue to control such defense of, as applicable) a Third Party Claim (i) unless it covenants to the Indemnified Party in writing within ten (10) Business Days after the Indemnified Party has given notice of the Third Party Claim to the Indemnifying Party to indemnify, defend and hold harmless the Indemnified Party from and against the entirety of any and all Losses that the Indemnified Party may suffer resulting from or arising out of the Third Party Claim (subject, however, to the limitations set forth in Section 6.6), (ii) in which equitable relief other than monetary damages is sought, (iii) if such Third Party Claim is brought by a Governmental Entity or is otherwise related to or arises in connection with any FDA, Tax or criminal or regulatory enforcement matter, (iv) if the Indemnified Party has been advised in writing by outside counsel that a legal conflict or potential legal conflict exists between the Indemnified Party and the Indemnifying Party in connection with conducting the defense of the Third Party Claim, (v) if settlement of, an adverse Judgment with respect to, or conduct of the defense of the Third Party Claim by the Indemnifying Party is, in the good faith judgment of the Indemnified Party, likely to be materially adverse to the Indemnified Party's or its Affiliates' reputation or continuing business interests (including its relationships with current or potential customers, licensors, distributors, suppliers, or other parties material to the conduct of its business), or (vi) the Indemnifying Party fails to diligently and vigorously and in good faith conduct the defense of the Third Party Claim.

(c) If the Indemnifying Party elects not to defend against, negotiate, settle or otherwise deal with any Third Party Claim that relates to any Losses indemnified against hereunder, or is not permitted to assume the defense (or, following such assumption of the defense in accordance herewith, is not permitted to continue to control such defense, as applicable) of a Third Party Claim pursuant to the proviso to the third sentence of Section 6.2(b), the Indemnified Party may defend against, negotiate, settle or otherwise deal with such Third Party Claim, subject to the provisions below. If the Indemnifying Party shall assume the defense of any Third Party Claim pursuant to the terms of this Agreement, the Indemnified Party may participate, at its own expense, in the defense of such Third Party Claim; *provided*, *however*, that such Indemnified Party shall be entitled to participate in any such defense with separate counsel at the expense of the Indemnifying Party if (i) so requested by the Indemnifying Party to participate or (ii) in the written opinion of outside counsel to the Indemnified Party a legal conflict or potential legal conflict exists between the Indemnified Party and the Indemnifying Party that would make such separate representation advisable. The Parties agree to reasonably cooperate with each other in connection with the defense, negotiation or settlement of any such Third Party Claim. Notwithstanding anything in this Section 6.2 to the contrary, the Indemnifying Party shall not, without the prior written consent of the Indemnified Party, settle or compromise any Third Party Claim or permit a default or consent to entry of any Judgment unless (A) the claimant provides to the Indemnified Party a full, general and unqualified release of the Indemnified Parties and their respective Affiliates and Representatives from all liability in respect of such Third Party Claim, (B) such settlement does not involve any injunctive relief binding upon the Indemnified Party or any of its Affiliates or Representatives, (C) such settlement does not create an Encumbrance upon any of the assets of any Indemnified Party or impose any restriction or condition that would apply to or materially affect any Indemnified Party or the conduct of any Indemnified Party's business, and (D) such

settlement does not involve any admission of liability or wrongdoing by any Indemnified Party or any of its Affiliates or Representatives.

6.3 Survival. The representations and warranties of Seller and Buyer under this Agreement, and liability for the breach thereof, shall survive the Effective Date and shall remain in full force and effect for a period of two (2) years following the Effective Date; *provided*, *however*, that all covenants (including Section 5.6), the Fundamental Representations and any claims for fraud shall survive the Effective Date and shall remain in full force and effect until the later of (a) ***[***]*** following the Effective Date and (b) the expiration of the applicable statute of limitations. No claim for breach of any representation, warranty, covenant or agreement may be brought after expiration of the survival periods set forth in this Section 6.3. Notwithstanding the foregoing, if written notice of a claim has been given in the manner required by Section 6.2 prior to the expiration of the applicable survival period by the Party seeking indemnification for such claim, then the relevant covenants, representations and warranties of the other Party shall survive as to such claim until such claim has been finally resolved pursuant to this ARTICLE 6.

6.4 Additional Indemnification Matters. The right of indemnification provided under this ARTICLE 6 shall not be affected by any knowledge acquired (or capable of being acquired) at any time, whether before or after the Effective Date, with respect to the accuracy or inaccuracy of, or compliance or noncompliance with, any representation, warranty, covenant, or agreement contained herein.

6.5 Adjustments. Any amount paid under this ARTICLE 6 shall be treated as an adjustment to the Purchase Price for all Tax purposes unless otherwise required by applicable Law.

6.6 Limitations of Liability.

(a) Notwithstanding anything to the contrary contained in this Agreement (but subject to Section 6.6(b) and Section 6.7), (i) each Party's maximum aggregate liability to the other Party arising out of or in any way related to this Agreement (including pursuant to this ARTICLE 6) shall not exceed an amount equal to the Purchase Price and (ii) except to the extent actually awarded against a Buyer or Seller Indemnified Party pursuant to a Judgment with respect to a Third Party Claim and except for a Party's fraud, then, in each case of this clause (ii), no Party shall have any liability under any provision of this Agreement for any punitive, incidental, consequential, special or indirect damages, including business interruption, diminution of value, loss of future revenue, profits or income, or loss of business reputation or opportunity relating to the breach or alleged breach of this Agreement and, in particular, no "multiple of profits" or "multiple of cash flow" or other valuation methodology will be used in calculating the amount of any Losses, regardless of the legal theory under which such liability or obligation may be sought to be imposed, whether sounding in contract or tort, or whether at law or in equity, or otherwise.

(b) Nothing in Section 6.6(a) shall operate to limit or exclude in any way Seller's liability for any and all Excluded Liabilities.

6.7 Remedies for Revocation. Notwithstanding anything in this Agreement to the contrary, but without limiting any of Buyer's rights and remedies under this Agreement, if the FDA revokes or otherwise invalidates any of the Purchased Assets for Seller's failure to Market

the Rare Pediatric Disease product for which the Priority Review Voucher was awarded within the Marketing Period (a "***Fundamental Breach Event***"), Seller shall promptly, and in any event not later than seven (7) days after the occurrence of such Fundamental Breach Event, pay Buyer an amount equal to the Purchase Price.

ARTICLE 7
GENERAL PROVISIONS

7.1 Notice Requirements. Any notice, request, demand, waiver, consent, approval, or other communication permitted or required under this Agreement shall be in writing, shall refer specifically to this Agreement and shall be deemed given only if (a) delivered by hand, (b) sent by internationally recognized overnight delivery service that maintains records of delivery, addressed to the Parties at their respective addresses specified in this Section 7.1 or to such other address as the Party to whom notice is to be given may have provided to the other Party in accordance with this Section 7.1, or (c) sent via email. Such notice shall be deemed to have been given (i) as of the date delivered by hand, (ii) on the second Business Day (at the place of delivery) after deposit with an internationally recognized overnight delivery service, or (iii) on the first Business Day following successful transmission via email.

If to Buyer, to:

[***]

with a copy (which shall not constitute notice) to:

[***]

If to Seller to:

Seller

X4 Pharmaceuticals, Inc.
61 North Beacon Street, 4th Floor
Boston, MA 02134
Attn: Paula Ragan, Ph.D. and Adam Mostafa
Email: paula.rogen@x4pharma.com and adam.mostafa@x4pharma.com

with a copy (which shall not constitute notice) to:

Goodwin Procter LLP
100 Northern Avenue
Boston, MA 02110
Attention: William D. Collins, Esq. and Gabriela Morales-Rivera, Esq.
Email: wcollins@goodwinlaw.com and gmoralesrivera@goodwinlaw.com

7.2 Construction. Except where the context otherwise requires, wherever used, the singular shall include the plural, the plural the singular, the use of any gender shall be applicable

to all genders and the word "or" is used in the inclusive sense (and/or). Whenever this Agreement refers to a number of days, unless otherwise specified, such number refers to calendar days. The captions of this Agreement are for convenience of reference only and in no way define, describe, extend, or limit the scope or intent of this Agreement or the intent of any provision contained in this Agreement. The term "including," "include," or "includes" as used herein shall mean "including, but not limited to," and shall not limit the generality of any description preceding such term. The words "will" and "shall" have the same meaning. The language of this Agreement shall be deemed to be the language mutually chosen by the Parties and no rule of strict construction shall be applied against either Party hereto. Each Party represents that it has been represented by legal counsel in connection with this Agreement and acknowledges that it has participated in the drafting hereof. In interpreting and applying the terms and provisions of this Agreement, the Parties agree that no presumption will apply against the Party which drafted such terms and provisions.

7.3 References. Unless otherwise specified, (a) references in this Agreement to any Article, Section, Schedule or Exhibit shall mean references to such Article, Section, Schedule or Exhibit of this Agreement, (b) references in any Section to any clause are references to such clause of such Section, and (c) references to any agreement, instrument, or other document in this Agreement refer to such agreement, instrument, or other document as originally executed or, if subsequently amended, replaced, or supplemented from time to time, as so amended, replaced, or supplemented and in effect at the relevant time of reference thereto.

7.4 Entire Agreement; Amendments. This Agreement, the documents, Exhibits, and Schedules referred to herein, and the Confidentiality Agreement sets forth and constitutes the entire agreement and understanding between the Parties with respect to the subject matter hereof and all prior agreements, understandings, promises, and representations, whether written or oral, with respect thereto are superseded hereby. Each Party confirms that it is not relying on any representations or warranties of the other Party except as specifically set forth in this Agreement. No amendment, modification, release, or discharge shall be binding upon the Parties unless in writing and duly executed by authorized Representatives of both Parties.

7.5 Assignment. Without the prior written consent of the other Party, neither Party shall sell, transfer, assign, delegate, pledge, or otherwise dispose of, whether voluntarily, involuntarily, by operation of law or otherwise, this Agreement or any of its rights or duties hereunder; *provided* that (a) either Party may make such a sale, transfer, assignment, delegation, pledge or other disposal without the other Party's consent to any of its Affiliates and (b) Buyer may make such a sale, transfer, assignment, delegation, pledge or disposal, in whole or in part, without Seller's consent, to any purchaser, transferee, or assignee of the Purchased Assets. With respect to any permitted assignment, the assigning Party shall remain responsible for the performance by such permitted assignee of the assigning Party's duties and obligations hereunder. Any attempted sale, transfer, assignment, delegation, pledge or other disposal in violation of this Section 7.5 shall be null and void and of no effect. All validly assigned and delegated rights and obligations of the Parties hereunder shall be binding upon and inure to the benefit of and be enforceable by and against the successors and permitted assigns of Buyer or Seller, as the case may be.

7.6 Severability. If any provision of this Agreement is held to be illegal, invalid, or unenforceable under any present or future law, and if the rights or obligations of either Party under this Agreement will not be materially and adversely affected thereby (a) such provision shall be fully severable; (b) this Agreement shall be construed and enforced as if such illegal, invalid, or unenforceable provision had never comprised a part hereof; (c) the remaining provisions of this Agreement shall remain in full force and effect and shall not be affected by the illegal, invalid, or unenforceable provision or by its severance herefrom; and (d) in lieu of such illegal, invalid, or unenforceable provision, there shall be added automatically as a part of this Agreement a legal, valid, and enforceable provision as similar in terms to such illegal, invalid, or unenforceable provision as may be possible and reasonably acceptable to the Parties. To the fullest extent permitted by applicable Law, each Party hereby waives any provision of Law that would render any provision hereof illegal, invalid, or unenforceable in any respect.

7.7 Governing Law. This Agreement or the performance, enforcement, breach or termination hereof shall be interpreted, governed by and construed in accordance with the laws of the State of Delaware, United States, excluding any conflicts or choice of law rule or principle that might otherwise refer construction or interpretation of this Agreement to the substantive law of another jurisdiction.

7.8 Submission to Jurisdiction. Each Party irrevocably agrees that any legal action or proceeding arising out of or relating to this Agreement brought by such Party or its successors or assigns shall be brought and determined in any Delaware state or federal court, and each Party hereby irrevocably (a) submits to the exclusive jurisdiction of the United States District Court in Wilmington, Delaware (or if such court does not have subject matter jurisdiction, a State Court of the State of Delaware located in Wilmington, Delaware) with regard to any such action or proceeding arising out of or relating to this Agreement and the transactions contemplated hereby and (b) agrees that service of any court paper may be made in the manner provided for in Section 7.1 or such other manner as may be provided under applicable Laws or court rules governing service of process. Each Party agrees not to commence any action, suit or proceeding relating thereto except in the courts described above in Delaware, other than actions in any court of competent jurisdiction to enforce any judgment, decree or award rendered by any such court in Delaware as described herein. Each Party hereby irrevocably and unconditionally waives, and agrees not to assert, by way of motion or as a defense, counterclaim or otherwise, in any action or proceeding arising out of or relating to this Agreement or the transactions contemplated hereby, (i) any claim that it is not personally subject to the jurisdiction of the courts in Delaware as described herein for any reason, (ii) that it or its property is exempt or immune from jurisdiction of any such court or from any legal process commenced in such courts (whether through service of notice, attachment prior to judgment, attachment in aid of execution of judgment, execution of judgment or otherwise), and (iii) that (A) the suit, action or proceeding in any such court is brought in an inconvenient forum, (B) the venue of such suit, action or proceeding is improper, or (C) this Agreement, or the subject matter hereof, may not be enforced in or by such courts.

7.9 WAIVER OF JURY TRIAL. EACH PARTY, TO THE EXTENT PERMITTED BY LAW, KNOWINGLY, VOLUNTARILY, AND INTENTIONALLY WAIVES ITS RIGHT TO A TRIAL BY JURY IN ANY ACTION OR OTHER LEGAL PROCEEDING ARISING OUT OF OR RELATING TO THIS AGREEMENT AND THE TRANSACTIONS IT

CONTEMPLATES. THIS WAIVER APPLIES TO ANY ACTION OR LEGAL PROCEEDING, WHETHER SOUNDING IN CONTRACT, TORT, OR OTHERWISE.

7.10 Waiver and Non-Exclusion of Remedies.

(a) Any term or condition of this Agreement may be waived at any time by the Party that is entitled to the benefit thereof, but no such waiver shall be effective unless set forth in a written instrument duly executed by or on behalf of the Party waiving such term or condition. The waiver by either Party hereto of any right hereunder or of the failure to perform or of a breach by the other Party shall not be deemed a waiver of any other right hereunder or of any other breach or failure by such other Party whether of a similar nature or otherwise, and nothing in this Agreement shall be deemed a waiver by any Party of any right to specific performance or injunctive relief. The rights and remedies provided herein are cumulative and do not exclude any other right or remedy provided by applicable Law or otherwise available except as expressly set forth herein.

(b) The Parties agree that irreparable harm would occur in the event that the transactions contemplated hereby are not consummated in accordance with the terms of this Agreement, and that money damages or other legal remedies would not be an adequate remedy for any such harm. Accordingly, the Parties acknowledge and hereby covenant and agree that in the event of any breach or threatened breach of the covenants, agreements, or obligations set forth in this Agreement, then in addition to any other remedy available at law or in equity, the non-breaching Party will be entitled to seek an injunction or injunctions to prevent or restrain any breaches or threatened breaches of this Agreement, and to specifically enforce the terms and provisions of this Agreement to enforce compliance with the covenants, agreements, and obligations under this Agreement. Each Party hereby covenants and agrees not to raise, and irrevocably waives, any objections to the availability of such relief that a remedy at law would be adequate and that a bond or other security will be required.

7.11 No Benefit to Third Parties. Except as provided in ARTICLE 6, the covenants and agreements set forth in this Agreement are for the sole benefit of the Parties and their successors and permitted assigns, and they shall not be construed as conferring any rights on any other Persons.

7.12 Counterparts; Execution. This Agreement may be executed in two (2) or more counterparts, each of which shall be deemed an original, but all of which together shall constitute one (1) and the same instrument. This Agreement may be executed by electronically transmitted signatures and such signatures shall be deemed to bind each Party hereto as if they were original signatures.

[SIGNATURE PAGE FOLLOWS]

IN WITNESS WHEREOF, each of Buyer and Seller has caused this Agreement to be executed and delivered by their respective officers thereunto duly authorized, all as of the date first written above.

BUYER

[***]

By: /s/ ***[***]***
Name: ***[***]***
Title: ***[***]***

IN WITNESS WHEREOF, each of Buyer and Seller has caused this Agreement to be executed and delivered by their respective officers thereunto duly authorized, all as of the date first written above.

SELLER

X4 PHARMACEUTICALS, INC.

By: /s/ Adam Mostafa
Name: Adam Mostafa
Title: Chief Financial Officer

Exhibit A

Approval Letter

Exhibit 2.3(b)

Form of Seller Cover Letter

[X4 Pharmaceuticals, Inc. Letterhead]

May 8, 2024

Transfer of Priority Review Voucher

[***]

[***]
Re: Xolremdi (mavorixafor) capsules
NDA 218709, SN:[●]
Transfer of Rare Pediatric Disease Priority Review Voucher PRV NDA 218709

Dear ***[***]***:

Reference is made to the New Drug Application (NDA) 218709 approval letter dated April 26, 2024 (the "***Approval Letter***") (Ref ID: 5371673) reflecting the grant of the Rare Pediatric Disease Priority Review Voucher PRV NDA 218709 (the "***Voucher***") to X4 Pharmaceuticals, Inc. ("X4") in connection with the U.S. Food and Drug Administration's ("***FDA's***") approval of NDA 218709 for XOLREMDI™ (mavorixafor) capsules.

Please be advised that, effective as of May 8, 2024, X4 has transferred complete ownership of the Voucher to ***[***]***, and ***[***]*** has legally accepted complete ownership of the Voucher from X4. X4 and ***[***]*** have exchanged letters acknowledging the transfer, copies of which are enclosed herein.

If you have any questions or need clarification regarding this submission, please do not hesitate to contact me using the following information:

Email: ***[***]***
Office/Cell: ***[***]***

Sincerely,

[***]

Exhibit 2.4(a)

Form of Bill of Sale

BILL OF SALE

This BILL OF SALE (the "***Bill of Sale***") is made and entered into as of May 8, 2024, by and between ***[***]*** ("***Buyer***") and X4 Pharmaceuticals, Inc., a Delaware corporation ("***Seller***"). Buyer and Seller may hereinafter be referred to individually as a "***Party***" and collectively as the "***Parties***". Reference is made to that certain Asset Purchase Agreement, dated as of May 8, 2024, by and between the Parties (the "***Purchase Agreement***"). Capitalized terms used but not otherwise defined herein shall have the meanings assigned to them in the Purchase Agreement.

WHEREAS, the Parties have entered into the Purchase Agreement, pursuant to which Seller has agreed to sell to Buyer, and Buyer has agreed to purchase from Seller, upon the terms and conditions set forth in the Purchase Agreement, all right, title, and interest of Seller in and to the Purchased Assets.

NOW, THEREFORE, in consideration of the premises and covenants hereinafter contained and the representations, warranties, and covenants contained in the Purchase Agreement, for good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, Buyer and Seller, intending to be legally bound, hereby agree as follows:

1. Effective Time. This Bill of Sale shall be effective as of the Effective Date.

2. Transfer of the Purchased Assets. Effective as of the Effective Date, pursuant to the terms and subject to the conditions of the Purchase Agreement, Seller (on behalf of itself and its Affiliates) hereby irrevocably sells, assigns, transfers, conveys and delivers to Buyer and its successors and its assigns, and Buyer hereby does purchase from Seller, all of Seller's and its Affiliates' right, title and interest in and to the Purchased Assets (including the Priority Review Voucher), in each case free and clear of all Encumbrances.

3. Binding Effect; Amendments. This Bill of Sale shall be binding upon, inure to the benefit of, and be enforceable by, the Parties and their respective legal representatives, successors and permitted assigns. Neither this Bill of Sale, nor any term or provision hereof, may be amended, modified, superseded, or cancelled except by an instrument in writing signed by each Party hereto.

4. Governing Law. This Bill of Sale or the performance, enforcement, breach or termination hereof shall be interpreted, and governed by the rules set forth in Sections 7.7, 7.8, and 7.9 of the Purchase Agreement. In the event of any conflict between the terms of this Bill of Sale and the Purchase Agreement, the Purchase Agreement shall control.

5. Counterparts. This Bill of Sale may be executed in two (2) or more counterparts, each of which shall be deemed an original, but all of which together shall constitute one (1) and the same instrument. This Bill of Sale may be executed by electronically transmitted signatures and such signatures shall be deemed to bind each Party hereto as if they were original signatures.

[SIGNATURE PAGE FOLLOWS]

IN WITNESS WHEREOF, each of Buyer and Seller has caused this Bill of Sale to be executed and delivered by their respective officers thereunto duly authorized, all as of the date first written above.

BUYER

[***]

By: _____
Name: ***[***]***
Title: ***[***]***

SELLER

X4 PHARMACEUTICALS, INC.

By: _____
Name: ***[***]***
Title: ***[***]***

Exhibit 2.4(b)

Form of Seller PRV Transfer Letter

[X4 Pharmaceuticals, Inc. Letterhead]

May 8, 2024

[***]
Re: NDA 218709 – Transfer of Rare Pediatric Disease Priority Review Voucher PRV NDA 218709 (the "***Voucher***")

Dear Sir or Madam:

Reference is made to the above-referenced NDA approval letter dated April 26, 2024 reflecting the grant of the Voucher to X4 Pharmaceuticals, Inc. ("***Seller***") in connection with the U.S. Food and Drug Administration's ("***FDA's***") approval of New Drug Application Number 218709 for XOLREMDI™ (mavorixafor) capsules.

Further, reference is made to that certain Asset Purchase Agreement, dated May 8, 2024 (the "***Agreement***") by and between ***[***]*** ("***Buyer***") and Seller. Pursuant to the Agreement, Seller has sold, transferred, assigned, conveyed and delivered the Voucher to Buyer, effective as of May 8, 2024 (the "***Effective Date***"), and Buyer has accepted complete ownership of the Voucher. This transfer is free and clear of all liens and provides Buyer with all of Seller's right, title, and interest in, to, and under the Voucher.

This letter acknowledges that Seller has irrevocably transferred ownership of the Voucher to Buyer, effective as of the Effective Date, and Buyer has legally accepted complete ownership of the Voucher from Seller.

This letter of transfer will be presented to the FDA by, or on behalf of, Buyer as evidence that Seller has transferred the Voucher to Buyer. Together with the acknowledgment of transfer letter from Buyer to Seller dated May 8, 2024, these letters serve as a complete record of transfer of the Voucher from Seller to Buyer.

Sincerely,

Adam Mostafa
Chief Financial Officer

Exhibit 2.5(c)

Form of Buyer PRV Transfer Letter

[***] Letterhead]

May 8, 2024

X4 Pharmaceuticals, Inc.
61 North Beacon Street, 4th Floor
Boston, MA 02134
Attn: ***[***]***

Re: NDA 218709 – Acknowledgment of Transfer of Rare Pediatric Disease Priority Review Voucher PRV NDA 218709 (the "***Voucher***")

Dear Sir or Madam:

Reference is made to the above-referenced NDA approval letter dated April 26, 2024 reflecting the grant of the Voucher to X4 Pharmaceuticals, Inc. ("***Seller***") in connection with the U.S. Food and Drug Administration's ("FDA's") approval of New Drug Application Number 218709 for XOLREMDI™ (mavorixafor) capsules.

Further, reference is made to that certain Asset Purchase Agreement, dated May 8, 2024 (the "***Agreement***") by and between ***[***]*** ("***Buyer***") and Seller. Pursuant to the Agreement, Seller has sold, transferred, assigned, conveyed and delivered the Voucher to the Buyer, effective as of May 8, 2024 (the "***Effective Date***"), and Buyer has accepted complete ownership of the Voucher. This transfer is free and clear of all liens and provides Buyer with all of Seller's right, title, and interest in, to, and under the Voucher.

This letter acknowledges and records that, as of the Effective Date, Buyer has legally accepted complete ownership of the Voucher from Seller.

This letter will be presented to the FDA by, or on behalf of, Buyer as evidence that Buyer acknowledges and accepts the transfer of the Voucher from Seller to Buyer. Together with the letter of transfer from Seller to Buyer dated May 8, 2024, these letters serve as a complete record of transfer of the Voucher from Seller to Buyer.

Sincerely,

_____

[***]

EXHIBIT 10.2

RULES AND CONDITIONS
FOR THE X4 PHARMACEUTICALS, INC.
NON-EMPLOYEE DIRECTORS' DEFERRED COMPENSATION PROGRAM
(THE "PROGRAM")

The following rules and conditions have been adopted by the Board of Directors of X4 Pharmaceuticals, Inc. (the "Company") to govern the deferral of Restricted Stock Units by non-employee directors of the Company (each, a "Non-Employee Director" and collectively, the "Non-Employee Directors") pursuant to the Company's Amended and Restated 2017 Equity Incentive Plan (as amended from time to time, the "Stock Plan") and the Company's Amended and Restated Director Compensation Policy (as amended from time to time, the "Policy"). Capitalized terms used but not defined herein shall have the meaning given such terms in the Stock Plan.

1. Election to Defer the Equity Retainer. A Non-Employee Director may elect in advance to defer the receipt of the initial and/or annual Restricted Stock Unit Grant made to such Non-Employee Director pursuant to the Policy under the Stock Plan (such grant, the "Equity Retainer"). To make such an election, except with respect to a newly elected or appointed Non-Employee Director, the Non-Employee Director must execute and deliver to the Company a deferral election form before the end of the calendar year preceding the calendar year in which the applicable Equity Retainer is scheduled to be granted. A newly elected or appointed Non-Employee Director, may, upon (but no later than 30 days after) becoming a Non-Employee Director, file a deferral election with respect to the initial Equity Retainer and/or to Equity Retainers that are awarded subsequent to the election. An election shall remain in effect from year to year until revoked in writing by the Non-Employee Director, but any revocation shall become effective only with respect to Equity Retainers that are granted in calendar years beginning after receipt and acceptance by the Company of a written revocation. All elections (including revocation thereof) must be made during an open window period while the Non-Employee Director is not in possession of any material non-public information relating to the Company.

2. Deferred Account. Upon the vesting of any Equity Retainer awarded to any Non-Employee Director who has elected to defer his or her Equity Retainer(s) pursuant to this Program, any shares of Common Stock that would otherwise have been issued to the Non-Employee Director upon such vesting shall be converted to deferred stock units on a one-to-one basis and credited to the Non-Employee Director's deferred account ("Account").

3. Dividend Equivalent Amounts. If dividends (other than dividends payable only in shares of Common Stock) are paid with respect to Common Stock, each Account shall be credited with a number of whole and fractional stock units determined by multiplying the dividend value per share by the stock unit balance of the Account on the record date and dividing the result by the fair market value of a share of Common Stock on the dividend payment date.

4. Period of Deferral. The deferred stock units in each Account shall be deferred until, and the period of deferral shall cease upon, the earliest of (a) the date a Non-Employee Director ceases to serve as a member of the Board of Directors of the Company and incurs a "separation from service" within the meaning of Section 409A of the Internal Revenue Code of 1986, as amended, and the regulations promulgated thereunder ("Section 409A"), (b) the consummation of a Reorganization Event (as defined in the Stock Plan) so long as such Reorganization Event

constitutes a "change in the ownership or effective control of the Company, or in the ownership of a substantial portion of the assets of the Company" within the meaning of Section 409A (a "Change in Control") or (c) the date of a Non-Employee Director's death.

5. Designation of Beneficiary. A Non-Employee Director may designate one or more beneficiaries to receive payments from his or her Account in the event of his or her death. A designation of beneficiary may apply to a specified percentage of a Non-Employee Director's entire interest in his or her Account. Such designation, or any change therein, must be in writing and shall be effective upon receipt by the Company. If there is no effective designation of beneficiary, or if no beneficiary survives the Non-Employee Director, the estate of the Non-Employee Director shall be deemed to be the beneficiary. All payments to a beneficiary or estate shall be made in a lump sum in shares of Common Stock, with any fractional share paid in cash.

6. Payment. All amounts credited to a Non-Employee Director's Account shall be paid in shares of Common Stock to the Non-Employee Director, or his or her designated beneficiary (or beneficiaries) or estate, in a single lump sum as soon as practicable (but in no event later than 30 days deferral) after the end of the first applicable period of deferral specified in Section 4 (above) occurs; provided, however, that fractional shares shall be paid in cash.

7. Adjustments. In the event of a stock dividend, stock split or similar change in capitalization affecting the Common Stock, the Company shall make appropriate adjustments in the number of stock units credited to the Non-Employee Directors' Accounts.

8. Non-transferability of Rights. During a Non-Employee Director's lifetime, any payment under this Program shall be made only to the Non-Employee Director. No sum or other interest under this deferred compensation arrangement shall be subject in any manner to anticipation, alienation, sale, transfer, assignment, pledge, encumbrance or charge, and any attempt by a Non-Employee Director or any beneficiary under this Program to do so shall be void. No interest under this deferred compensation arrangement shall in any manner be liable for or subject to the debts, contracts, liabilities, engagements or torts of a Non-Employee Director or beneficiary entitled thereto. Notwithstanding the foregoing, the Company may make payments to an individual other than a Non-Employee Director to the extent required by a domestic relations order.

9. Company's Obligations to Be Unfunded and Unsecured. The Accounts maintained under this Program shall at all times be entirely unfunded, and no provision shall at any time be made with respect to segregating assets of the Company (including Common Stock) for payment of any amounts hereunder. No Non-Employee Director or other person shall have any interest in any particular assets of the Company (including Common Stock) by reason of the right to receive payment under this Program, and any Non-Employee Director or other person shall have only the rights of a general unsecured creditor of the Company with respect to any rights under this Program.

10. Section 409A. This Program is intended to be a compliant deferred compensation plan under Section 409A and shall be administered in accordance with the requirements of Section 409A.

11. Incorporation of Plan. This Program shall be subject to the terms and conditions of the Stock Plan and the Policy. Capitalized terms in this document shall have the meaning specified in the Stock Plan, unless a different meaning is specified herein.

Adopted as of June 28, 2024 (the "Effective Date")

EXHIBIT 10.3

X4 PHARMACEUTICALS, INC.
AMENDED AND RESTATED INSIDER TRADING POLICY
(AS OF JUNE 28, 2024)

INTRODUCTION

During the course of your relationship with X4 Pharmaceuticals, Inc. (the "***Company***"), you may receive material information that is not yet publicly available ("***material nonpublic information***" or "***MNPI***") about the Company or other publicly traded companies with which the Company has business relationships. MNPI may give you, or someone you pass that information on to, an informational "***leg up***" over others when deciding whether to buy, sell or otherwise transact in the Company's securities or the securities of another publicly traded company. This policy sets forth guidelines with respect to transactions in the Company's securities, in each case by all our employees, directors and consultants who are advised that they are subject to this policy ("**designated consultants**"), and all other related persons subject to this policy as described below (collectively referred to in this Policy as "***Covered Persons***" or "***you***").

STATEMENT OF POLICY

It is the policy of the Company that any Covered Person who is aware of material nonpublic information relating to the Company may not, directly or indirectly:

1. engage in any transaction in the Company's securities, except as otherwise specified under the heading "***Exceptions to this Policy***" below;

2. recommend the purchase or sale of any Company securities;

3. disclose material nonpublic information to persons within the Company whose jobs do not require them to have that information, or outside of the Company to other persons, such as family, friends, business associates and investors, unless the disclosure is made in accordance with the Company's policies regarding the protection or authorized external disclosure of information regarding the Company; or

4. assist anyone engaged in the above activities.

The prohibition against insider trading is absolute. It applies ***even if*** the decision to trade is not based on such material nonpublic information. It also applies to transactions that may be necessary or justifiable for independent reasons (such as the need to raise money for an emergency expenditure) and also to very small transactions. All that matters is whether you are aware of any MNPI relating to the Company at the time of the transaction.

The U.S. federal securities laws do not recognize any mitigating circumstances to insider trading. In addition, even the appearance of an improper transaction must be avoided to preserve the Company's reputation for adhering to the highest standards of conduct. In some circumstances, you may need to forgo a planned transaction even if you planned it before becoming aware of the material nonpublic information. So, even if you believe you may suffer

an economic loss or sacrifice an anticipated profit by waiting to trade, you must not engage in any trading.

It is also important to note that the laws prohibiting insider trading are not limited to trading by the insider alone; providing material nonpublic information to others (and/or advising others on the basis of material nonpublic information) so that they may trade, known as "tipping," is illegal and squarely prohibited by this policy. Liability in such cases can extend both to the "tippee"—the person to whom the insider disclosed material nonpublic information—and to the "tipper," the insider himself or herself. In such cases, you can be held liable for your own transactions, as well as the transactions by a tippee and even the transactions of a tippee's tippee. For these and other reasons, no Covered Person may disclose material nonpublic information to persons within the Company whose jobs do not require them to have that information, or outside of the Company to other persons (unless the disclosure is made in accordance with the Company's policies regarding the external disclosure of Company information).

In addition, this policy's prohibitions against insider trading and tipping also apply to trading in securities of certain other companies. Specifically, whenever, during the course of your service to or employment by the Company, you become aware of material nonpublic information about another company (1) with which the Company has an existing business relationship, including but not limited to, the Company's distributors, vendors, customers or suppliers or collaboration, marketing, research, development or licensing partners, or (2) with which the Company is in active discussions concerning a potential transaction or business relationship, no Covered Person or Related Persons (defined below) may trade in any securities of that company, give trading advice about that company, tip or disclose that information, pass it on to others or engage in any other action to take advantage of that information. If your work regularly involves handling or discussing confidential information of one of our partners, suppliers or customers, you should consult with the Chief Financial Officer, General Counsel or Chief Executive Officer before trading in that company's securities. Additionally, if you believe you may be in possession of nonpublic information about the Company that could potentially have a material effect on the stock price of a company with which the Company does not have an existing business relationship or with which the Company is not discussing a potential transaction or business relationship, you should exercise caution when trading in the securities of that company as the SEC has successfully brought an insider trading claim against an employee in those circumstances.

There are no exceptions to this policy, except as specifically noted above or below.

TRANSACTIONS SUBJECT TO THIS POLICY

This policy applies to all transactions in securities issued by the Company, as well as derivative securities that are not issued by the Company, such as exchange-traded put or call options or swaps relating to the Company's securities. Accordingly, for purposes of this policy, the terms "***trade,***" "***trading,***" and "***transactions***" include not only purchases and sales of the Company's common stock in the public market but also any other purchases, sales, transfers or other acquisitions and dispositions of its common or preferred equity, options, warrants and other securities (including debt securities) and other arrangements or transactions that affect economic exposure to changes in the prices of these securities.

PERSONS SUBJECT TO THIS POLICY

This policy applies to you as a Covered Person and all other employees, directors and designated consultants of the Company and its subsidiaries. This policy also applies to members of your immediate family, persons with whom you share a household, persons who are your economic dependents, all persons who execute trades on your behalf, all trusts, family partnerships and other types of entities formed for your benefit or for the benefit of a member of your family and over which you have the ability to influence or direct investment decisions concerning securities and all investment funds, trusts, retirement plans, partnerships, corporations and other types of entities over which you have the ability to influence or direct investment decisions concerning securities; provided, however, that this policy do not apply to any such entity that engages in the investment of securities in the ordinary course of its business (e.g., an investment fund or partnership) if the entity has established its own insider trading controls and procedures in compliance with applicable securities laws and it (or an affiliated entity) has represented to the Company that its affiliated entities: (a) engage in the investment of securities in the ordinary course of their respective businesses; (b) have established insider trading controls and procedures in compliance with securities laws; and (c) are aware the securities laws prohibit any person or entity who has material nonpublic information concerning the Company from purchasing or selling securities of the Company or from communicating such information to any other person under circumstances in which it is reasonably foreseeable that such person is likely to purchase or sell securities. The foregoing persons who are deemed subject to this policy are referred to in this policy as "***Related Persons.***" *You are responsible for making sure that your Related Persons comply with this policy*.

MATERIAL NONPUBLIC INFORMATION

MATERIAL INFORMATION

It is not always easy to figure out whether you are aware of material nonpublic information. But there is one important factor to determine whether nonpublic information you know about a public company is material: whether the information could be expected to affect the market price of that company's securities or to be considered important by investors who are considering trading that company's securities. If the information makes you want to trade, it would probably have the same effect on others. Keep in mind that both positive and negative information can be material.

There is no bright-line standard for assessing materiality; rather, materiality is based on an assessment of all of the facts and circumstances and is often evaluated by relevant enforcement authorities with the benefit of hindsight. Depending on the specific details, the following items may be considered material nonpublic information until publicly disclosed within the meaning of this policy. There may be other types of information that would qualify as material information as well; use this list merely as a non-exhaustive guide:

- financial results or forecasts;
- status of product or product candidate development or regulatory approvals;
- clinical data relating to products or product candidates;
- timelines for pre-clinical studies or clinical trials;

- communications with government agencies, including, but not limited to, the U.S. Food and Drug Administration;
- strategic plans;
- acquisitions or dispositions of assets, divisions or companies;
- public or private sales of debt or equity securities;
- stock splits, dividends or changes in dividend policy;
- the establishment of a repurchase program for the Company's securities;
- gain or loss of a significant licensor, licensee or supplier;
- changes or new corporate partner relationships or collaborations;
- New major contracts, orders or suppliers, or the loss of any of them;
- notice of issuance or denial of patents;
- regulatory developments;
- management, member of the Company's Board of Directors or control changes;
- employee layoffs;
- a disruption in the Company's operations, supply chain or breach or unauthorized access of its property or assets, including its facilities and information technology infrastructure (such as cybersecurity risks and incidents);
- tender offers or proxy fights;
- accounting restatements;
- litigation or settlements; and
- impending bankruptcy.

WHEN INFORMATION IS CONSIDERED PUBLIC

The prohibition on trading when you have material nonpublic information lifts once that information becomes publicly disseminated. For information to be considered publicly disseminated, it must be widely disseminated through a press release, a filing with the Securities and Exchange Commission (the "***SEC***"), or other widely disseminated announcement. Once information is publicly disseminated, it is still necessary to afford the investing public with sufficient time to absorb the information. Generally speaking, information will be considered publicly disseminated for purposes of this policy only after one full trading day has elapsed since the information was publicly disclosed. For example, if we announce material nonpublic information before trading begins on Wednesday, then you may execute a transaction in our securities on Thursday; if we announce material nonpublic information after trading ends on Wednesday, then you may execute a transaction in our securities on Friday. Depending on the particular circumstances, the Company may determine that a longer or shorter waiting period should apply to the release of specific material nonpublic information.

QUARTERLY TRADING BLACKOUTS

To minimize even the appearance of insider trading, we have established "***quarterly trading blackout periods***" during which certain Covered Persons—regardless of whether they are aware of MNPI—may not conduct any trades in the Company's securities. Specifically, Section 16 officers, members of the Board of Directors, any employee with the position Vice President or higher, and all employees in the Company's finance, legal, investor relations, and sales departments (collectively, "Quarterly Blackout Covered Persons") will be subject to quarterly trading blackout periods. That means that, except as described in this policy, all

Quarterly Blackout Covered Persons and their Related Persons will be able to trade in the Company's securities only during limited open trading window periods that generally will begin after one full trading day has elapsed since the public dissemination of the Company's annual or quarterly financial results and end at the beginning of the next quarterly trading blackout period as described below. Of course, even during an open trading window period, you may not (unless an exception applies) conduct any trades in the Company's securities if you are otherwise in possession of MNPI.

For purposes of this policy, each "***quarterly trading blackout period***" will generally begin at the end of the day that is the last day of each fiscal quarter and end after one full trading day has elapsed since the public dissemination of the Company's financial results for that quarter. Please note that the quarterly trading blackout period may commence early or may be extended if, in the judgment of the Chief Financial Officer (or, if there is no Chief Financial Officer, our General Counsel), there exists undisclosed information that would make trades by the Company's employees, directors and designated consultants inappropriate. In such circumstances, all Quarterly Blackout Covered Persons will be informed of any changes to the quarterly trading blackout period; it is important to note that the fact that the quarterly trading blackout period has commenced early or has been extended should be considered material nonpublic information that should not be communicated to any other person.

A Quarterly Blackout Covered Person who believes that special circumstances require him or her to trade during a quarterly trading blackout period should consult the Chief Financial Officer (or, if there is no Chief Financial Officer, our General Counsel) or (if the Chief Financial Officer or General Counsel is seeking permission to trade), the Chief Executive Officer. Permission to trade during a quarterly trading blackout period will be granted, at the sole discretion of the Chief Financial Officer, General Counsel or Chief Executive Officer, only where the circumstances are extenuating, the General Counsel (or, as applicable in accordance with the preceding sentence, the Chief Financial Officer or Chief Executive Officer) concludes that the person is not in fact aware of any material nonpublic information relating to the Company or its securities, and there appears to be no significant risk that the trade may subsequently be questioned.

EVENT-SPECIFIC TRADING BLACKOUTS

From time to time, an event may occur that is material to the Company and is known by only a few directors, officers and/or employees. So long as the event remains material and nonpublic, the persons designated by the Chief Financial Officer (or, if there is no Chief Financial Officer, our General Counsel) may not trade in the Company's securities. In that situation, the Company will notify the designated individuals (and by extension, their Related Persons) that they may not trade in the Company's securities. The existence of an event-specific trading blackout should also be considered material nonpublic information and should not be communicated to any other person. Even if you have not been designated as a person who should not trade due to an event-specific trading blackout, you should not trade while aware of material nonpublic information. Exceptions will not be granted during an event-specific trading blackout.

The quarterly and event-driven trading blackouts do not apply to those transactions to which this policy does not apply, as described under the heading "***Exceptions to the Policy***" below.

EXHIBIT 10.3

REQUIREMENTS FOR 10B5-1 TRADING AND PRE-CLEARANCE OF TRANSACTIONS

SECTION 16 OFFICERS MUST TRADE ONLY PURSUANT TO RULE 10B5-1 PLANS

In accordance with the Company's Rule 10b5-1 Trading Guidelines, and subject to the exceptions below, ***Covered Persons who are also Section 16 Officers and members of the Company's Board of Directors, are required to establish a trading plan under Rule 10b5-1 to govern all transactions that they make in the Company's securities***. All other Covered Persons may, but are not required to, establish a trading plan under Rule 10b5-1.

PRE-CLEARANCE AND ADVANCE NOTICE OF TRANSACTIONS

In addition to the requirements above, all employees who are Vice President level or above ("***Covered Insiders***") face a further restriction: Even during an open trading window, they may not engage in any transaction in the Company's securities without first obtaining written pre-clearance of the transaction from the Company's Chief Financial Officer (or, if there is no Chief Financial Officer, our General Counsel) or his or her designee (the "Clearing Officer") at least two business days in advance of the proposed transaction by entering such preclearance request in the Company's equity plan administration software (Shareworks by Morgan Stanley). The Clearing Officer will then determine whether the transaction may proceed and, if so, will direct the Compliance Coordinator (as identified in the Company's Section 16 Compliance Program) to help comply with any required reporting requirements under Section 16(a) of the Exchange Act. Pre-cleared transactions not completed within five business days will require new pre-clearance. The Company may choose to shorten this period. The Clearing Officer is under no obligation to approve a transaction submitted for pre-clearance and may determine not to permit the transaction. If a Covered Person seeks preclearance and permission to engage in the transaction is denied, then he or she should refrain from initiating any transaction in Company securities and should not inform any other person of the restriction.

Persons subject to pre-clearance must also give advance notice of their plans to exercise an outstanding stock option to the Compliance Coordinator. Once any transaction takes place, the officer, director or applicable member of management must immediately notify the Compliance Coordinator and any other individuals identified under the heading "Notification of Execution of Transaction" in the Section 16 Compliance Program so that the Company may assist in any Section 16 reporting obligations.

EXCEPTIONS TO THE POLICY

This policy does not apply in the case of the following transactions, except as specifically noted:

1. **Option Exercises**. This policy does not apply to the mere exercise of options granted under the Company's equity compensation plans for cash or, where permitted under the option, by a net exercise transaction with the Company or by delivery to the Company of already-owned Company stock. This policy does, however, apply to any sale of stock as part of a

broker-assisted cashless exercise or any other market sale, whether or not for the purpose of generating the cash needed to pay the exercise price or pay taxes.

2. Tax Withholding Transactions. This policy does not apply to the surrender of shares directly to the Company to satisfy tax withholding obligations as a result of the issuance of shares upon vesting or exercise of restricted stock units, options or other equity awards granted under the Company's equity compensation plans. Nor does this policy apply to any non-discretionary market sale of the stock received upon exercise or vesting of any such equity awards, as mandated by the Company's Board of Directors, the sole purpose of generating the cash needed to pay the exercise price or pay taxes.

3. ESPP. This policy does not apply to the purchase of stock by employees under the Company's Employee Stock Purchase Plan ("***ESPP***") on periodic designated dates in accordance with the ESPP. This policy does, however, apply to any sale of stock acquired pursuant to the ESPP.

4. 10b5-1 Automatic Trading Programs. Under Rule 10b5-1 of the Securities Exchange Act of 1934, as amended ("***Exchange Act***"), and as permitted by the Company, employees, directors and designated consultants may establish a trading plan under Rule 10b5-1 of the Securities Exchange Act of 1934, where a broker is instructed to buy and sell the Company's securities based on pre-determined criteria (a "***10b5-1 Trading Plan***"). As noted above, Section 16 officers of the Company are requested to establish a 10b5-1 Trading Plan in order to transact in securities of the Company. So long as a 10b5-1 Trading Plan is properly established, purchases and sales of the Company's securities pursuant to that Trading Plan are not subject to this policy. To be properly established, a 10b5-1 Trading Plan must comply with the requirements of Rule 10b5-1 of the Exchange Act and the Company's Rule 10b5-1 Trading Guidelines. Moreover, all 10b5-1 Trading Plans must be reviewed and approved by the Company so that the Company can confirm that the 10b5-1 Trading Plan complies with all pertinent company policies and applicable securities laws.

5. Gifts. This policy does not apply to bona fide gifts of the Company's securities that have been pre-cleared by the Company's Chief Financial Officer (or, if there is no Chief Financial Officer, our General Counsel) or his or her designee. Whether a gift is truly bona fide will depend on the facts and circumstances surrounding each gift. Pre-clearance must be obtained at least two business days in advance of the proposed gift, and pre-cleared gifts not completed within five business days will require new pre-clearance. The Company may choose to shorten this period.

6. 401(k) Plan. This policy does not apply to purchases of the Company's securities in the Company's 401(k) plan resulting from your periodic contribution of money to the plan pursuant to your payroll deduction election. This policy does apply, however, to certain elections you may make under the 401(k) plan, including: (a) an election to increase or decrease the percentage of your periodic contributions that will be allocated to the Company's stock fund; (b) an election to make an intra-plan transfer of an existing account balance into or out of the Company's stock fund; (c) an election to borrow money against your 401(k) plan account if the loan will result in a liquidation of some or all of your Company stock fund balance; and (d) an election to pre-pay a plan loan if the pre-payment will result in allocation of loan proceeds to the Company's stock fund.

EXHIBIT 10.3

SPECIAL AND PROHIBITED TRANSACTIONS

1. Inherently Speculative Transactions. No Covered Person may engage in short sales, transactions in short sales, transactions in put options, call options or other derivative securities on an exchange or in any other organized market, or in any other inherently speculative transactions with respect to the Company's stock.

2. Hedging Transactions. Hedging or monetization transactions can be accomplished through a number of possible mechanisms, including through the use of financial instruments such as prepaid variable forwards, equity swaps, collars and exchange funds. Such hedging transactions may permit a Company employee, director or designated consultant to continue to own the Company's securities obtained through employee benefit plans or otherwise, but without the full risks and rewards of ownership. When that occurs, the Company's employee, director or designated consultant may no longer have the same objectives as the Company's other stockholders. Therefore, the Company's employees, directors and designated consultants are prohibited from engaging in any such transactions. The purchase of or the entry into other transactions that are designed to offset or "***hedge***" decreases in the market value of the Company's securities may permit a Covered Person to continue to own the Company's securities obtained through employee benefit plans or otherwise, but without the full risks and rewards of ownership. When that occurs, the Covered Person may no longer have the same objectives as the Company's other stockholders. Therefore, the Covered Persons are prohibited from engaging in any such transactions.

Hedging can be accomplished through any number of possible mechanisms, including through the use of financial instruments such as prepaid variable forwards, equity swaps, collars and exchange funds. Other examples include borrowing or other arrangements involving a non-recourse pledge of the Company's securities and selling a security future that establishes a position that increases in value as the value of the underlying equity security decreases.

3. Margin Accounts and Pledged Securities. Securities held in a margin account as collateral for a margin loan may be sold by the broker without the customer's consent if the customer fails to meet a margin call. Similarly, securities pledged (or hypothecated) as collateral for a loan may be sold in foreclosure if the borrower defaults on the loan. Because a margin sale or foreclosure sale may occur at a time when the pledgor is aware of material nonpublic information or otherwise is not permitted to trade in the Company's securities, Covered Persons are prohibited from holding Company Securities in a margin account or otherwise pledging the Company's securities as collateral for a loan.

4. Standing and Limit Orders. Standing and limit orders (except standing and limit orders under approved Trading Plans, as discussed above) create heightened risks for insider trading violations similar to the use of margin accounts. There is no control over the timing of purchases or sales that result from standing instructions to a broker, and as a result the broker could execute a transaction when a Covered Person is in possession of material nonpublic information. The Company therefore discourages placing standing or limit orders on the Company's securities. If a Covered Person determines that they must use a standing order or limit order (other than under an approved Trading Plan as discussed above), the order should be limited to short duration (e.g., two trading days) and the person using such standing order or limit order is required to cancel such instructions immediately in the event restrictions are

imposed on their ability to trade pursuant to the "***Quarterly Trading Blackouts***" and "***Event-Specific Trading Blackouts***" provisions above.

SHORT-SWING TRADING, CONTROL STOCK AND SECTION 16 REPORTS

Officers and directors subject to the reporting obligations under Section 16 of the Exchange Act should take care to avoid short-swing transactions (within the meaning of Section 16(b) of the Exchange Act) and the restrictions on sales by control persons (Rule 144 under the Securities Act of 1933, as amended), and are responsible for ensuring that they (or the Company on their behalf) file all appropriate Section 16(a) reports (Forms 3, 4 and 5) which are described in the Section 16 Compliance Program and any notices of sale required by Rule 144.

CONTROL SHARES AND SHORT-SWING TRADING

Officers and directors may also be subject to restrictions on sales of shares by control persons (Rule 144 under the U.S. Securities Act of 1933, as amended). In addition, officers and directors will be subject to the reporting obligations under Section 16 of the Exchange Act will also be subject to profit disgorgement on short-swing transactions (within the meaning of Section 16(b) of the Exchange Act). Officers and directors should take care not to violate these rules and to file any notices of sale required by Rule 144.

PROHIBITION OF TRADING DURING PENSION PLAN BLACKOUTS

No director or executive officer of the Company may, directly or indirectly, purchase, sell or otherwise transfer any equity security of the Company (other than an exempt security) during any "blackout period" (as defined in Regulation BTR under the Exchange Act) if a director or executive officer acquires or previously acquired such equity security in connection with his or her service or employment as a director or executive officer. This prohibition does not apply to any transactions that are specifically exempted, including but not limited to, purchases or sales of the Company's securities made pursuant to, and in compliance with, a Trading Plan; compensatory grants or awards of equity securities pursuant to a plan that, by its terms, permits executive officers and directors to receive automatic grants or awards and specifies the terms of the grants and awards; or acquisitions or dispositions of equity securities involving a *bona fide* gift or by will or the laws of descent or pursuant to a domestic relations order. The Company will notify each director and executive officer of any blackout periods in accordance with the provisions of Regulation BTR. Because Regulation BTR is very complex, no director or executive officer of the Company should engage in any transactions in the Company's securities, even if believed to be exempt from Regulation BTR, without first consulting with the Chief Financial Officer (or, if there is no Chief Financial Officer, our General Counsel).

POLICY'S DURATION

This policy continues to apply to your transactions in the Company's securities or the securities of other applicable public companies engaged in business transactions with the Company even after your relationship with the Company has ended. If you are aware of material nonpublic information when your relationship with the Company ends, you may not trade the Company's securities or the securities of other applicable companies until the material nonpublic information has been publicly disseminated or is no longer material. Further, if you leave the

Company during a trading blackout period, then you may not trade the Company's securities or the securities of other applicable companies until the trading blackout period has ended.

INDIVIDUAL RESPONSIBILITY

Persons subject to this policy have ethical and legal obligations to maintain the confidentiality of information about the Company and to not engage in transactions in the Company's securities or the securities of other applicable public companies while aware of material nonpublic information. Each individual is responsible for making sure that he or she complies with this policy, and that any family member, household member or other person or entity whose transactions are subject to this policy, as discussed under the heading "***Persons Subject to this Policy***" above, also comply with this policy. In all cases, the responsibility for determining whether an individual is aware of material nonpublic information rests with that individual, and any action on the part of the Company or any employee or director of the Company pursuant to this policy (or otherwise) does not in any way constitute legal advice or insulate an individual from liability under applicable securities laws. You could be subject to severe legal penalties and disciplinary action by the Company for any conduct prohibited by this policy or applicable securities laws. See "***Penalties***" below.

PENALTIES

Anyone who engages in insider trading or otherwise violates this policy may be subject to both civil liability and criminal penalties. Violators also risk disciplinary action by the Company, including termination of employment. Anyone who has questions about this policy should contact their own attorney or the Company's Chief Financial Officer (or, if there is no Chief Financial Officer, our General Counsel). Please also see Frequently Asked Questions, which are attached as **EXHIBIT A**.

WAVERS

A waiver of any provision of this policy or the procedures discussed herein may be authorized in writing by the Audit Committee of the Board of Directors ("**Audit Committee**"), or the Company's Board of Directors (the "**Board of Directors**"). All waivers shall be reported to the Board of Directors.

AMENDMENTS

The Company is committed to continuously reviewing and updating its policies and procedures. The Board of Directors therefore reserve the right to amend, alter or terminate this policy at any time and for any reason. A current copy of the Company's policies regarding insider trading may be obtained at https://x4pharma.sharepoint.com/sites/Finance-Public.

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: August 8, 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: August 8, 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 June 30, 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 8th day of August, 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)*