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DELTA REPORT

10-Q

XFOR - X4 PHARMACEUTICALS, INC

10-Q - MARCH 31, 2024 COMPARED TO 10-Q - SEPTEMBER 30, 2023

The following comparison report has been automatically generated

TOTAL DELTAS	3037
CHANGES	160
DELETIONS	2176
ADDITIONS	701

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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 September 30, 2023 March 31, 2024

or

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

For the transition period from _____ to _____

Commission File Number: 001-38295

X4 PHARMACEUTICALS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**27-3181608**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
61 North Beacon Street, 4th Floor Boston, Massachusetts	**02134**
(Address of principal executive offices)	**(Zip Code)**

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

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value $0.001 per share	**XFOR**	**The Nasdaq Stock Market LLC**

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

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company.

See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
		Emerging growth company	☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of November 7, 2023 May 3, 2024, the registrant had 167,291,209 167,937,781 shares of common stock, $0.001 par value per share, outstanding.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended (the “Securities Act”), and Section 21E of the Securities Exchange Act of 1934, as amended, or the (“Exchange Act”), that relate to future events or to our future operations or financial performance. All statements, other than statements of historical facts, contained in this Quarterly Report on Form 10-Q, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward-looking statements. These statements may be identified by such forward-looking terminology as “may,” “should,” “expects,” “intends,” “plans,” “anticipates,” “believes,” “estimates,” “predicts,” “potential,” “continue” or the negative of these terms or other comparable terminology. Our forward-looking statements are based on a series of expectations, assumptions, estimates and projections about our company, are not guarantees of future results or performance and involve substantial risks and uncertainty. We may not actually achieve the plans, intentions or expectations disclosed in these forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in these forward-looking statements. These forward-looking statements are subject to a number of known and unknown risks, uncertainties and assumptions, including risks described in the section titled “Risk Factors” and elsewhere in this report, regarding, among other things:

- the initiation, timing, progress and results of our current and future preclinical studies and clinical trials and related preparatory work and the period during which the results of the trials will become available, as well as our research and development programs;
- the potential benefits, including clinical utility, that may be derived from any of our products or product candidates;
- the timing of and our ability to obtain and maintain regulatory approval of our existing product or product candidates or any product candidates that we may develop in the future, and any related restrictions, limitations, or warnings in the label of any approved product candidates;
- our plans to research, develop, manufacture and commercialize our product or product candidates;
- the timing of our regulatory filings for our product candidates, along with regulatory developments in the United States and other foreign countries;
- the size and growth potential of the markets for our products and product candidates, if approved, and the rate and degree of market acceptance of our products and product candidates, including reimbursement that may be received from payors;
- the benefits of U.S. Food and Drug Administration (“FDA”) and European Commission designations, including, without limitation, Fast Track, Orphan Drug and Breakthrough Therapy;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our ability to attract and retain qualified employees and key personnel;
- our competitive position and the development of and projections relating to our competitors or our industry;
- our expectations regarding our ability to obtain and maintain intellectual property protection;
- the success of competing therapies that are or may become available;
- our estimates and expectations regarding future operations, financial position, revenues, costs, expenses, uses of cash, capital requirements or our need for additional financing;
- our ability to continue as a going concern;
- our plans to in-license, acquire, develop and commercialize additional product candidates;
- the impact of laws and regulations;
- our plans to identify additional product candidates with significant commercial potential that are consistent with our commercial objectives;
- our ability to raise additional capital;
- our strategies, prospects, plans, expectations or objectives; and

- other risks and uncertainties, including those listed under the section titled “Risk Factors” in this Quarterly Report.

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

SUMMARY OF SELECTED RISKS ASSOCIATED WITH OUR BUSINESS

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

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

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

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

- We depend almost entirely on the success of our commercial product, XOLREMDI™, which has been approved for use as an oral, once-daily therapy to increase the number of circulating mature neutrophils and lymphocytes in patients 12 years of age and older with WHIM (warts, hypogammaglobulinemia, infections, and myelokathexis) syndrome in the U.S., and on our lead product candidate, mavorixafor, which we are developing for the potential treatment of other chronic neutropenic disorders, including WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) syndrome and, contingent on a potential strategic partnerships, for the treatment of Waldenström’s. 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 including WHIM syndrome, is smaller than we anticipate, our potential future revenue from mavorixafor for the treatment of any of the diseases may be adversely affected and our business may suffer.

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

- 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.

- 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.

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

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

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

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

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

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

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

- Our employees, principal investigators, CROs and consultants may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements, which could have a material adverse effect on our business.
- We may depend on such collaborations for the development and commercialization of our product candidates. If those collaborations are not successful, we may not be able to capitalize on the market potential of our product candidates.
- If we are unable to protect our intellectual property rights, our competitive position could be harmed.
- Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.
- Our future success depends on our ability to retain executives and to attract, retain and motivate key personnel in a competitive environment for skilled biotechnology personnel.
- We will need to grow the size of our organization, and we may experience difficulties in managing this growth.
- Our term load loan contains restrictions that limit our flexibility in operating our business.
- Our business could be adversely affected by economic downturns, inflation, increases in interest rates, natural disasters, public health crises, such as the COVID-19 pandemic, political crises, geopolitical events, such as the wars in Ukraine and Israel, Gaza, or other macroeconomic conditions, which have in the past and may in the future negatively impact our business and financial performance.
- Our stock price has been and is likely to continue to be volatile and fluctuate substantially.

PART I FINANCIAL INFORMATION

Item 1. FINANCIAL STATEMENTS.

X4 PHARMACEUTICALS, INC.

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

		September 30, 2023	December 31, 2022			
	March 31, 2024				March 31, 2024	December 31, 2023
Assets	**Assets**					
Current assets:	Current assets:					
Current assets:						
Current assets:						
Cash and cash equivalents						
Cash and cash equivalents						
Cash and cash equivalents	Cash and cash equivalents	$131,581	$121,718			
Marketable securities	Marketable securities	10,102	—			
Research and development incentive receivable	Research and development incentive receivable	393	1,152			

Prepaid expenses and other current assets	Prepaid expenses and other current assets	5,796	5,807
Total current assets	Total current assets	147,872	128,677
Property and equipment, net	Property and equipment, net	772	1,104
Goodwill	Goodwill	17,351	17,351
Right-of-use assets	Right-of-use assets	6,054	7,229
Other assets	Other assets	1,244	1,225
Total assets	Total assets	$173,293	$155,586
Liabilities and Stockholders' Equity	**Liabilities and Stockholders' Equity**		
Current liabilities:	Current liabilities:		
Current liabilities:			
Current liabilities:			
Accounts payable			
Accounts payable			
Accounts payable	Accounts payable	$ 8,132	$ 7,777
Accrued expenses	Accrued expenses	16,184	12,034
Current portion of lease liability	Current portion of lease liability	1,116	1,198
Current portion of long-term debt		—	1,315
Total current liabilities	Total current liabilities	25,432	22,324
Long-term debt, net of discount and current portion		54,322	32,304
Total current liabilities			
Total current liabilities			
Long-term debt, including accretion, net of discount			
Lease liabilities	Lease liabilities	2,848	3,603
Warrant liability (Note 4)	Warrant liability (Note 4)	22,014	23,131
Other liabilities	Other liabilities	1,083	173
Total liabilities	Total liabilities	105,699	81,535
Commitments and contingencies (Note 9)	Commitments and contingencies (Note 9)		Commitments and contingencies (Note 9)
Stockholders' equity:	Stockholders' equity:		

Common stock, $0.001 par value, 500,000,000 shares authorized as of September 30, 2023 and December 31, 2022, respectively; 164,705,712 and 121,667,250 shares issued and outstanding as of September 30, 2023 and December 31, 2022, respectively	165	122
Common stock, $0.001 par value, 500,000,000 shares authorized as of March 31, 2024 and December 31, 2023, respectively; 167,937,781 and 167,434,595 shares issued and outstanding as of March 31, 2024 and December 31, 2023, respectively		
Common stock, $0.001 par value, 500,000,000 shares authorized as of March 31, 2024 and December 31, 2023, respectively; 167,937,781 and 167,434,595 shares issued and outstanding as of March 31, 2024 and December 31, 2023, respectively		
Common stock, $0.001 par value, 500,000,000 shares authorized as of March 31, 2024 and December 31, 2023, respectively; 167,937,781 and 167,434,595 shares issued and outstanding as of March 31, 2024 and December 31, 2023, respectively		
Additional paid-in capital	526,323	450,786
Accumulated other comprehensive loss	(119)	(119)
Accumulated deficit	(458,775)	(376,738)
Total stockholders' equity	67,594	74,051
Total liabilities and stockholders' equity	$173,293	$155,586

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

X4 PHARMACEUTICALS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(In thousands, except share and per share amounts)

(Unaudited)

Three Months Ended March 31,		
Three Months Ended March 31,		
Three Months Ended March 31,		
2024		

	2024			
	2024			
	Three Months Ended September 30,		Nine Months Ended September 30,	
Operating expenses:				
	2023	2022	2023	2022
Operating expenses:				
Operating expenses:	Operating expenses:			
Research and development	Research and development	$ 19,081	$ 14,110	56,745	42,044
Research and development				
Research and development				
Selling, general and administrative	Selling, general and administrative	8,133	6,044	25,578	20,457
Gain on sale of non-financial asset		—	—	—	(509)
Selling, general and administrative				
Selling, general and administrative				
Total operating expenses				
Total operating expenses				
Total operating expenses	Total operating expenses	27,214	20,154	82,323	61,992
Loss from operations	Loss from operations	(27,214)	(20,154)	(82,323)	(61,992)
Other income (expense), net:					
Loss from operations					
Loss from operations					
Other (expense) income, net:					
Other (expense) income, net:					
Other (expense) income, net:					
Interest income					
Interest income					
Interest income	Interest income	1,388	14	3,137	21
Interest expense	Interest expense	(1,634)	(1,018)	(3,891)	(2,849)
Change in fair value of derivative liability		—	—	—	511
Interest expense					
Interest expense					
Change in fair value of warrant liability	Change in fair value of warrant liability	25,164	—	743	—
Other income (expense), net		17	(441)	342	(440)

Total other income (expense), net	24,935	(1,445)	331	(2,757)
Change in fair value of warrant liability				
Change in fair value of warrant liability				
Other income, net				
Other income, net				
Other income, net				
Total other (expense) income, net				
Total other (expense) income, net				
Total other (expense) income, net				
Loss before provision for income taxes				
Loss before provision for income taxes				
Loss before provision for income taxes	Loss before provision for income taxes	(2,279)	(21,599)	(81,992)	(64,749)
Provision for income taxes	Provision for income taxes	26	(13)	45	14
Net loss and comprehensive loss		(2,305)	(21,586)	(82,037)	(64,763)
Deemed dividend on Class B Warrant price reset		—	(287)	—	(2,546)
Net loss attributable to common stockholders		$ (2,305)	$ (21,873)	$ (82,037)	$ (67,309)
Net loss per share attributable to common stockholders—basic and diluted		$ (0.01)	$ (0.26)	$ (0.48)	$ (1.32)
Weighted average shares of common stock outstanding—basic and diluted		196,988	83,211	170,751	50,976
Provision for income taxes					
Provision for income taxes					
Net loss					
Net loss					
Net loss					
Net loss per share: basic and diluted					
Net loss per share: basic and diluted					
Net loss per share: basic and diluted					
Weighted average shares of common stock outstanding: basic and diluted					
Weighted average shares of common stock outstanding: basic and diluted					
Weighted average shares of common stock outstanding: basic and diluted					
Other comprehensive loss, net of tax:					
Other comprehensive loss, net of tax:					
Other comprehensive loss, net of tax:					

Net loss						
Net loss						
Net loss						
Change in unrealized loss related to available-for-sale debt securities						
Change in unrealized loss related to available-for-sale debt securities						
Change in unrealized loss related to available-for-sale debt securities						
Comprehensive loss						
Comprehensive loss						
Comprehensive loss						

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, 2022	121,667,250	$ 122	$450,786	$ (119)	$ (376,738)	$ 74,051

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2023						
Vesting of restricted stock units	Vesting of restricted stock units 540,238		—			—
Stock-based compensation expense			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 shares of common stock under employee stock purchase plan		114,577	—	175			175
Exercise of stock options and warrants		7,476,345	7	8,804			8,811
Vesting of restricted stock units							
Vesting of restricted stock units	Vesting of restricted stock units	98,555	—	—			—
Stock-based compensation expense	Stock-based compensation expense			2,142			2,142
Unrealized loss on marketable securities							
Net loss	Net loss					(55,712)	(55,712)
Balance at June 30, 2023		164,418,011	164	523,960	(119)	(456,470)	67,535
Vesting of restricted stock units		286,201	1				1
Exercise of stock options and warrants		1,500		2			2
Stock-based compensation expense				2,361			2,361
Net loss						(2,305)	(2,305)
Balance at September 30, 2023		**164,705,712**	**$ 165**	**$526,323**	**$ (119)**	**$ (458,775)**	**$ 67,594**
Balance at March 31, 2024							

	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

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Net loss					(24,020)	(24,020)
Balance at March 31, 2023	122,207,488	$ 122	$ 452,431	$ (119)	$ (400,758)	$ 51,676

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, 2021	28,127,657	$ 28	$ 347,374	$ (119)	$ (282,871)	$ 64,412
Issuance of common stock, redeemable common stock and pre-funded warrants for the purchase of common stock, net of issuance costs	2,512,902	3	5,817			5,820
Exercise of warrants	100		—			—
Vesting of restricted stock units, net of shares withheld and retired to satisfy tax obligations	168,817		(12)			(12)
Stock-based compensation			1,459			1,459
Net loss					(21,965)	(21,965)
Balance at March 31, 2022	30,809,476	31	354,638	(119)	(304,836)	49,714
Issuance of common stock under employee stock purchase plan	72,727		49			49
Vesting of restricted stock units	108,995		—			—
Stock-based compensation			1,522			1,522
Net loss					(21,212)	(21,212)
Balance at June 30, 2022	30,991,198	31	356,209	(119)	(326,048)	30,073
Issuance of common stock and prefunded warrants for the purchase of common stock, net of issuance costs	37,649,086	38	13,459			13,497
Vesting of restricted stock units	94,269					—
Reclassification of warrant liability to equity			38,754			38,754
Stock-based compensation expense			1,111			1,111
Net loss					(21,586)	(21,586)
Balance at September 30, 2022	68,734,553	$ 69	$ 409,533	$ (119)	$ (347,634)	$ 61,849

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)

	Nine Months Ended September 30,

	2023	2022
	Three Months Ended March 31,	
2024		
Cash flows from operating activities:		
Net loss	$ (82,037)	$ (64,763)
Net loss		
Net loss		
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense		
Stock-based compensation expense		
Stock-based compensation expense	6,148	4,092
Depreciation and amortization expense	357	389
Non-cash lease expense	1,173	1,104
Accretion of debt discount	681	653
Change in fair value of warrant liability	(743)	—
Other	(46)	255
Changes in operating assets and liabilities:		

	Three Months Ended March 31,	
2024	2023	
Cash flows from operating activities:		
Net loss	$ (64,763)	
Net loss		
Net loss		
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense		
Stock-based compensation expense		
Stock-based compensation expense	4,092	
Depreciation and amortization expense	389	
Non-cash lease expense	1,104	
Accretion of debt discount	653	
Change in fair value of warrant liability	—	
Other	255	
Changes in operating assets and liabilities:		

Prepaid expenses, other current assets and research and development incentive receivable	Prepaid expenses, other current assets and research and development incentive receivable	459	158
Prepaid expenses, other current assets and research and development incentive receivable			
Prepaid expenses, other current assets and research and development incentive receivable			
Accounts payable	Accounts payable	426	(481)
Accrued expenses and other long-term liabilities	Accrued expenses and other long-term liabilities	5,651	1,305
Lease liabilities	Lease liabilities	(834)	(721)
Net cash used in operating activities	Net cash used in operating activities	(68,765)	(58,009)
Cash flows from investing activities:	**Cash flows from investing activities:**		
Purchase of marketable securities	Purchase of marketable securities	(11,025)	—
Purchase of marketable securities			
Purchase of marketable securities			
Sales and maturities of marketable securities	Sales and maturities of marketable securities	1,000	—
Acquisition of property and equipment	Acquisition of property and equipment	(25)	(69)

Net cash used in investing activities	Net cash used in investing activities	(10,050)	(69)
Cash flows from financing activities:	**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		8,615	70
Employee taxes paid related to net share settlement of vested restricted stock units		—	(12)
Fees paid to amendment loan and security agreement and issuance costs related to the sale of warrants			
Fees paid to amendment loan and security agreement and issuance costs related to the sale of warrants			
Fees paid to amendment loan and security agreement and issuance costs related to the sale of warrants	Fees paid to amendment loan and security agreement and issuance costs related to the sale of warrants	(631)	(3,300)
Repayments of borrowings and accrued end-of-term fees under loan and security agreement	Repayments of borrowings and accrued end-of-term fees under loan and security agreement	(2,064)	(795)
Proceeds from borrowings under loan and security agreement		22,500	—
Proceeds from sale of common stock, warrants and pre-funded warrants, net of issuance costs	Proceeds from sale of common stock, warrants and pre-funded warrants, net of issuance costs	59,999	60,623
Net cash provided by financing activities		88,419	56,586

Proceeds from sale of common stock, warrants and pre-funded warrants, net of issuance costs			
Proceeds from sale of common stock, warrants and pre-funded warrants, net of issuance costs			
Net cash used in financing activities			
Effect of exchange rate changes on cash, cash equivalents and restricted cash	Effect of exchange rate changes on cash, cash equivalents and restricted cash	(28)	(468)
Net increase (decrease) in cash, cash equivalents and restricted cash		9,576	(1,960)
Net decrease in cash, cash equivalents and restricted cash			
Cash, cash equivalents and restricted cash at beginning of period	Cash, cash equivalents and restricted cash at beginning of period	123,028	83,108
Cash, cash equivalents and restricted cash at end of period	Cash, cash equivalents and restricted cash at end of period	$132,604	$ 81,148
Issuance costs not yet paid related to sale of shares of common stock, warrants and pre-funded warrants		$ —	$ 22

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

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

1. NATURE OF THE BUSINESS AND BASIS OF PRESENTATION

X4 Pharmaceuticals, Inc. (together with its subsidiaries, the “Company”) is a late-stage clinical biopharmaceutical companydiscovering, developing, and developing 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. The On April 29, 2024, the Company announced that the FDA approved

the Company's lead clinical candidate is New Drug Application ("NDA") for mavorixafor, a small-molecule antagonist of the chemokine receptor CXCR4 that which is being developed marketed under the trade name XOLREMDI™, for use as an oral, once-daily therapy. 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 mature, functional white blood cells from the bone marrow into the bloodstream, the Company believes that mavorixafor has the potential to provide therapeutic benefit across a variety of chronic neutropenic immune system disorders and WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) syndrome ("WHIM"), a rare, primary immunodeficiency. Following announcement of positive top-line data from the Company's pivotal, global, Phase 3 clinical trial in November 2022, the Company submitted a New Drug Application ("NDA") addition to the U.S. Food and Drug Administration ("FDA") in August 2023, seeking approval of oral, once-daily mavorixafor in the treatment of people aged 12 years and older with WHIM syndrome. The FDA accepted the NDA on October 30, 2023 for Priority Review, establishing As a PDUFA/action date of April 30, 2024. As the Company prepares for a potential launch of mavorixafor for WHIM in the U.S. in the second quarter of 2024, result, the Company is also enrolling participants in conducting a Phase 2 clinical trial evaluating the safety and efficacy of mavorixafor as a monotherapy and in combination with human granulocyte colony-stimulating factor ("G-CSF") in people with certain chronic neutropenic disorders. X4 Interim data from this Phase 2 trial are expected to be presented in June 2024.The Company also expects plans to initiate a pivotal, global Phase 3 clinical trial of mavorixafor in the first half second quarter of 2024 that aims to evaluate the efficacy, safety, and tolerability of oral once-daily mavorixafor with or without G-CSF in certain people with congenital or acquired primary autoimmune and idiopathic chronic neutropenic disorders. 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 Accounting Standards Update ("ASU") No. 2014-15,*Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern (Subtopic 205-40)* ("ASU 2014-15"), the 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 November 9, 2023, which is the date that the condensed consolidated financial statements were are issued. Although the Company has an approved drug product, sales of the Company's drug product over the next 12 months will not be sufficient to fund the Company's operating expenses. Since inception, the Company has incurred significant operating losses and negative cash flows from operations. As of September 30, 2023 March 31, 2024, the Company had $141.7 million $80.9 million of cash, cash equivalents and short-term marketable securities, and an accumulated deficit of $458.8 million $529.7 million. Net cash used in operating activities was $68.8 million $33.6 million for the nine three months ended September 30, 2023 March 31, 2024. On August 2, 2023, the The Company entered into an amendment (the "Amendment") to has a covenant under its Second Amended and Restated Loan and Security Agreement (as amended by the Amendment, the (the "Hercules Loan Agreement") with Hercules Capital Inc. ("Hercules"). The Amendment extended the interest-only period from September 2024 to March 2025, provided an additional $22.5 million in borrowings at closing and increased the available borrowing capacity to $115.0 million. The Hercules Loan Agreement , that requires that the Company currently maintain a minimum level of cash of $20 million through January 2025 and thereafter , subject to reductions upon adjustments beginning January 31, 2025 to 20% of outstanding borrowings. Based on its current cash flow projections, which excludes any new capital raising activities and the potential sale of the Priority Review Voucher that was granted by the FDA concurrent with the approval of XOLREMDI as discussed below, the Company believes it would not maintain the minimum cash required to satisfy this covenant beginning in the first quarter of 2025. In such event, the lender could require the repayment of all outstanding debt. Accordingly, management has concluded that the Company's achievement accumulated deficit, history of operational milestones.

Management has assessed losses, future expected losses and negative cash flows met the ASC 205-40 standard for raising substantial doubt about the Company's ability to continue as a going concern in accordance with the requirements of Accounting Standards Codification ("ASC") 205-40. concern. The Company has does not have adequate financial resources to fund its forecasted operating costs for at least one year after the date that these condensed consolidated financial statements were issued on November 9, 2023. are issued. The accompanying consolidated financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might result from the outcome of this uncertainty. Accordingly, the condensed consolidated financial statements have been prepared on a basis that assumes the Company will continue as a going concern and which contemplates the realization of assets and satisfaction of liabilities and commitments in the ordinary course of business.

Based on Concurrent with its current approval of XOLREMDI and pursuant to its Rare Pediatric Disease designation, the FDA granted the Company a Priority Review Voucher ("PRV") that may be used to obtain Priority Review for a subsequent application or sold to another drug sponsor. The Company's cash flow projections exclude any potential sale of any PRV to a third party and considering include a $7.0 million milestone payment triggered by the terms achievement of the Hercules Loan Agreement and with no additional funding, the Company believes it has sufficient cash, cash equivalents and marketable securities to fund operations into 2025. However, to such approval as discussed in Note 3. To finance its operations in 2025 and beyond, the Company will need to raise additional capital, which cannot be assured. Unless and until the Company reaches profitability in the future, it will require additional capital to fund our its operations, which could be raised through a combination of equity offerings, debt financings, other third-party funding, marketing and distribution arrangements and other collaborations and strategic alliances. If the Company is unable to obtain funding, it could be forced to delay, reduce

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

The Company is subject to risks common to companies in the biopharmaceutical industry including, but not limited to, uncertainties relating to conducting preclinical and clinical research and development, the manufacture and supply of products and product candidates for clinical and commercial use, obtaining and maintaining regulatory approvals and pricing and reimbursement for the Company's products and product candidates, market acceptance, managing global growth and operating expenses, availability of additional capital, competition, obtaining and enforcing patents, stock price volatility, dependence on collaborative relationships and third-party service providers, dependence on key personnel, and from time to time government investigations, litigation, and potential product liability claims.

Principles of Consolidation—The condensed 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, ("X4 Austria"), and X4 Therapeutics, Inc. All significant intercompany accounts and transactions have been eliminated.

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

Unaudited Interim Condensed Consolidated Financial Statements— The condensed consolidated balance sheet at December 31, 2022 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, 2022 December 31, 2023 included in the 2022 2023 Annual Report filed with the SEC on March 21, 2023 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 nine months ended September 30, 2023 March 31, 2024 are not necessarily indicative of the results of operations that may be expected for the year ending December 31, 2023 December 31, 2024.

Use of Estimates— The preparation of the Company's condensed consolidated financial statements in conformity with GAAP U.S. Generally Accepted Accounting Principles ("GAAP") requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the consolidated financial statements, and the reported amounts of expenses during the reporting period. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to, the accrual of research and development expenses, and the impairment or lack of impairment of long-lived assets including operating lease right-of-use assets and goodwill, and assumptions underlying the fair value of warrant liabilities. 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 condensed 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, 2022 December 31, 2023 filed with the SEC on March 21, 2023 March 21, 2024. Since the date of those consolidated financial statements, there have been no material changes to the Company's significant accounting policies.

Cash and Cash Equivalents— The Company considers all highly liquid investments with maturities of 90 days or less at the date of purchase to be cash equivalents. Cash equivalents consisted of money market funds, treasury bills and federal government agency notes as of September 30, 2023 and December 31, 2022.

Marketable Securities— Marketable securities consist of short-term securities classified as available-for-sale having maturities greater than 90 days, but less than 365 days from the date of acquisition. The Company determines the appropriate classification of the securities at the time they are acquired and evaluate the appropriateness of such classifications at each balance sheet date. The Company's marketable securities consist of available-for-sale securities that are classified as Level 2 because their value is based on valuations using significant inputs derived from, or corroborated by, observable market data. The cost of available-for-sale securities sold is based on the specific-identification method. Unrealized gain and losses on available-for-sale are included as a component of other comprehensive (loss) income on the condensed consolidated balance sheets and condensed consolidated statements of stockholders' equity and as a component of total comprehensive (loss) income on the condensed consolidated statement of operations and comprehensive loss until realized. Realized gains and losses on the sale of marketable securities are determined using the specific-identification method and recorded in other (expense) income, net on the accompanying condensed consolidated statements of operations and comprehensive loss. The Company reviews marketable securities for impairment whenever the fair value of a marketable security is less than the amortized cost and evidence indicates that a marketable security's carrying amount is not recoverable. Unrealized losses are evaluated for impairment under ASC 326, *Financial Instruments - Credit Losses*, to determine if the impairment is credit-related or noncredit-related. Credit-related impairment is recognized as an allowance on the condensed consolidated balance sheets with a

corresponding adjustment to earnings, and noncredit-related impairment is recognized in other comprehensive (loss) income, net of taxes. Evidence considered in this assessment includes reasons for the impairment, compliance with our investment policy, the severity of the impairment, collectability of the security, and any adverse conditions specifically related to the security, an industry, or geographic area.

Restricted Cash

(in thousands)	(in thousands)	As of September 30, 2023	As of December 31, 2022	(in thousands)	As of March 31, 2024	As of December 31, 2023
Letter of credit security: Waltham lease						
Letter of credit security: Waltham lease						
Letter of credit security: Waltham lease	Letter of credit security: Waltham lease	$ 250	$ 250			
Letter of credit security: Vienna Austria lease	Letter of credit security: Vienna Austria lease	202	205			
Letter of credit security: Boston lease	Letter of credit security: Boston lease	571	855			
Total restricted cash	Total restricted cash	$ 1,023	$ 1,310			
Restricted cash included in prepaid expenses and other current assets	Restricted cash included in prepaid expenses and other current assets	$ 250	$ 285			
Restricted cash included in other assets	Restricted cash included in other assets	$ 773	$ 1,025			

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

The following table provides a reconciliation of cash, cash equivalents, and restricted cash reported within the condensed consolidated balance sheets to the sum of to the total of amounts shown in the Company's condensed consolidated statements of cash flows as of September 30,

2023 March 31, 2024 and December 31, 2022 December 31, 2023:

(in thousands)	(in thousands)	September 30, 2023	December 31, 2022
(in thousands)			
(in thousands)			
Cash and cash equivalents	Cash and cash equivalents	$ 131,581	$ 121,718
Restricted cash, current portion		250	285
Cash and cash equivalents			
Cash and cash equivalents			
Restricted cash, current (included within prepaid expenses and other current assets)			
Restricted cash, current (included within prepaid expenses and other current assets)			
Restricted cash, current (included within prepaid expenses and other current assets)			
Restricted cash, non-current			
Restricted cash, non-current			
Restricted cash, non-current	Restricted cash, non-current	773	1,025
Total cash, cash equivalents and restricted cash	Total cash, cash equivalents and restricted cash	$ 132,604	$ 123,028
Total cash, cash equivalents and restricted cash			
Total cash, cash equivalents and restricted cash			

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 September 30, 2023 March 31, 2024 that necessitated an interim impairment test of goodwill.

Recently Adopted Accounting Standards

In June 2016, November 2023, the Financial Accounting Standards Board ("FASB") issued ASU 2016-13, 2023-07, *Credit Losses* *Segment Reporting (Topic 326)* *Measurement of Credit Losses on Financial Instruments* *Improvements to Reportable Segment Disclosures* ("ASU 2016-

13” 2023-07”), as amended. . Among other disclosure enhancements, ASU 2016-13 2023-07 requires that financial assets measured at amortized cost, entities with one reportable segment, such as trade receivables, be presented net of expected credit losses, which may be estimated based on relevant the Company, disclose general information for its reportable segment, such as historical experience, current conditions, the title and future expectation position of the individual identified as the Chief Operating Decision Maker (“CODM”), which for each pool of similar financial asset. The new guidance requires enhanced disclosures related to trade receivables and associated credit losses. The Company adopted ASU 2016-13 on January 1, 2023. As the Company did not have accounts receivable is the Chief Executive Officer, the types of products and services provided by the reportable segment, the measure of profit or marketable securities on 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 balance sheet financial statements as of and for the date three years ending December 31, 2024 and must be adopted retrospectively. Although the Company continues to evaluate the potential impact of adoption, there was no impact to ASU 2023-07, the Company does not believe that the adoption of ASU 2016-13. 2023-07 will have a material impact on its consolidated financial statement when adopted.

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

3. LICENSE, COLLABORATION AND FUNDING AGREEMENTS

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

3. LICENSE, COLLABORATION AND FUNDING AGREEMENTS

Research and Development Incentive Program

The Company participates in a research and development incentive program provided by the Austrian government whereby the Company is entitled to reimbursement by the Austrian government for a percentage of qualifying research and development expenses and capital expenditures incurred by the Company’s subsidiary in Austria. As of September 30, 2023 March 31, 2024, the amount due under the program is $0.4 million $0.7 million, which amount is included in research and development incentive receivable in the condensed consolidated balance sheet. During each of the nine three months ended September 30, 2023 March 31, 2024 and 2022, 2023, the Company recorded $0.4 million $0.2 million and $0.1 million of income related to the program within the condensed consolidated statements of operations and comprehensive loss as other income.

License and Collaboration Agreements

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

The As of March 31, 2024, the Company is obligated to pay Genzyme make future milestone payments in the aggregate amount of up to $25.0 million, $20.0 million, contingent upon the achievement by the Company of certain clinical-stage regulatory and sales milestones with respect to

licensed products. During A $7.0 million regulatory milestone became payable 30 days following the nine months ended September 30, 2023, Company's receipt of FDA approval of the Company accrued Company's NDA on April 26, 2024. The remaining regulatory milestones include (i) $3.0 million for the acceptance by the European Medicines Agency ("EMA") of the Company's first drug application and (ii) $5.0 million related to a development milestone under upon the Genzyme Agreement as notification by the Company believes that it is probable under ASC Topic 450, *Contingencies*, that the milestone will be achieved. The milestone was achieved on October 30, 2023. The $5.0 million accrued payment has been recorded within research and development expense on the condensed consolidated statements of operations. An additional $7.0 million EMA of regulatory approval of the Company's first drug application. The Company must also make one-time sales milestone payments are not yet probable but are reasonably possible of becoming payable with the next eight to nine month period under the Genzyme Agreement. $0.5 million, $1.5 million and $3.0 million on cumulative net sales of $50.0 million, $150.0 million and $300.0 million, respectively.

The Company is also obligated to pay Genzyme tiered royalties based on net sales of licensed products that the Company commercializes under the agreement.

Gain on SaleUpon the first sale of Non-Financial Asset
During the nine months ended September 30, 2022, a third party, who had previously acquired rights to certain intellectual property from Company's drug product in the Company, terminated the arrangement and transferred these rights back to the Company. During the nine months ended September 30, 2022 U.S., the Company transferred these rights will incur a royalty on annual net sales at a rate of 6% up to another third party in return for $0.5 million. $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 has no continuing involvement will include these royalties in any ongoing research and development activities associated with the intellectual property. The Company concluded that these third parties are "non-customers" as the underlying intellectual property transferred to and from these third parties supports potential drug candidates that are not aligned with the Company's strategic focus and, therefore, are not an output cost of the Company's ordinary activities. Accordingly, the Company accounted for the sale of the intellectual property as the sale of a non-financial asset under ASC Topic 610-20, *Gains and Losses from the Derecognition of Nonfinancial Assets* ("ASC 610-20"), and included the gain in gain on sale of non-financial asset for the nine months ended September 30, 2022. goods sold.

There were no material modifications of the Company's license or collaboration agreements during the nine three months ended September 30, 2023 March 31, 2024.

4. FAIR VALUE OF FINANCIAL ASSETS AND LIABILITIES

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

	Fair Value Measurements as of September 30, 2023 Using:				Fair Value Measurements as of March 31, 2024 Using:				
(in thousands)	(in thousands)	Level 1	Level 2	Level 3	Total	(in thousands)	Level 1	Level 2	Level 3	Total
Assets:	Assets:									

Cash equivalents—money market funds	$98,119	$ —	$ —	$ 98,119
Marketable securities—treasury bills, federal government agency notes	—	10,102	—	10,102
	$98,119	$10,102	$ —	$108,221

Cash equivalents—money market funds and U.S. Treasury bills

Cash equivalents—money market funds and U.S. Treasury bills

Cash equivalents—money market funds and U.S. Treasury bills

Marketable securities—U.S. Treasury notes, U.S. Treasury bills, and federal government agency notes

$

Liabilities:				
Embedded derivative liability	$ —	$ —	$ 10	$ 10
Class C warrant liability	—	—	22,014	22,014
	$ —	$ —	$22,024	$ 22,024

Embedded derivative liability

Embedded derivative liability

Class C warrant liability (Note 10)

$

	Fair Value Measurements as of December 31, 2022 Using:			
(in thousands)	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents—money market funds	$70,170	$2,858	$ —	$73,028
	$70,170	$2,858	$ —	$73,028

	Fair Value Measurements as of December 31, 2023 Using:			
(in thousands)	Level 1	Level 2	Level 3	Total
Liabilities:				
Embedded derivative liability	$ —	$ —	$ 10	$ 10
Class C warrant liability	—	—	23,131	23,131
	$ —	$ —	$23,141	$23,141

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

The Company's cash equivalents consisted of money market funds invested primarily in short term commercial paper, asset- backed U.S. Treasury securities certificate of deposits and repurchase agreements. direct investments in U.S. Treasury securities. The money market funds were valued based on reported market pricing quoted prices in active markets for the identical assets, which represents a Level 1 measurement, or measurement. U.S. Treasury securities were valued by using inputs observable in active markets for similar securities, which represents a Level 2 measurement. The Company has an investment portfolio of federal government agency notes and treasury bills. These investments are measured at measurement in the fair value using Level 2 assumptions. hierarchy.

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

The following table provides a roll-forward of the aggregate fair values financial instruments for which fair values are determined using Level 3 inputs:

(in thousands)	Embedded Derivative Liability	Class C Warrant Liability	Total
Balance as of December 31, 2022	$ 10	$ 23,131	$ 23,141
Change in fair value	—	(743)	(743)
Reclassification of warrant liability to permanent equity upon exercise	—	(374)	(374)
Balance as of September 30, 2023	$ 10	$ 22,014	$ 22,024

(in thousands)	Embedded Derivative Liability	Class C Warrant Liability	Total
Balance as of December 31, 2023	$ 10	$ 15,683	$ 15,693
Change in fair value	—	13,755	13,755
Balance as of March 31, 2024	$ 10	$ 29,438	$ 29,448

Valuation of Embedded Derivative Liability— The fair value of the embedded derivative liability recognized in connection with the Company's loan agreement with Hercules (see Note 7), which is associated with additional fees due to Hercules upon events of default, was determined based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy. The fair value of this embedded derivative liability, which is reported within other non-current liabilities on the condensed consolidated balance sheets, is

estimated by the Company at each reporting date based, in part, on the results of third party third-party valuations, which are were prepared based on a discounted cash flow model that considers 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 14% 17%.

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

The Company calculated the fair value of the Class C Warrants using the Black-Scholes option pricing model, which represents a Level 3 measurement within the fair value hierarchy, with the following inputs:

	September 30, 2023	December 31, 2022		March 31, 2024	December 31, 2023
Common stock price	Common stock price	$1.09	$0.99	Common stock price	$1.39	$0.84
Risk-free interest rate	Risk-free interest rate	4.6 %	4.0 %	Risk-free interest rate	4.3 %	3.9 %
Expected term (in years)	Expected term (in years)	4.2	4.9	Expected term (in years)	3.7	3.9
Expected volatility	Expected volatility	91.3 %	101.7 %	Expected volatility	96.1 %	96.2 %
Expected dividend yield	Expected dividend yield	— %	— %	Expected dividend yield	— %	— %

5. PROPERTY AND EQUIPMENT, NET

Property and equipment, net consisted of the following:

(in thousands)	(in thousands)	September 30, 2023	December 31, 2022	(in thousands)	March 31, 2024	December 31, 2023

Leasehold improvements	Leasehold improvements	$ 228	$ 228
Furniture and fixtures	Furniture and fixtures	1,273	1,268
Computer equipment	Computer equipment	181	173
Software	Software	24	24
Lab equipment	Lab equipment	651	639
		2,357	2,332
	2,411		
Less: Accumulated depreciation and amortization	Less: Accumulated depreciation and amortization	(1,585)	(1,228)
		$ 772	$ 1,104
	$		

Depreciation and amortization expense related to property and equipment was $357 $62 thousand and $389 $127 thousand for the nine three months ended September 30, 2023 March 31, 2024 and 2022, 2023, respectively.

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

6. ACCRUED EXPENSES

Accrued expenses consisted of the following:

(in thousands)	(in thousands)	September 30, 2023	December 31, 2022	(in thousands)	March 31, 2024	December 31, 2023
Accrued employee compensation and benefits	Accrued employee compensation and benefits	$ 6,067	6,592			
Accrued external research and development expenses	Accrued external research and development expenses	8,563	3,906			
Accrued professional fees	Accrued professional fees	976	571			

Accrued deferred financing fees		—	591
Other	Other	578	374
		$16,184	$12,034

Other	
Other	
	$

7. LONG-TERM DEBT

Long-term debt consisted of the following:

(in thousands)	(in thousands)	September 30, 2023	December 31, 2022
Principal amount of long-term debt	Principal amount of long-term debt	$55,000	$32,500
Debt discount, net of accretion	Debt discount, net of accretion	(1,007)	(196)
Cumulative accretion of end of term payments	Cumulative accretion of end of term payments	329	1,315
Long-term debt	Long-term debt	$54,322	$33,619
Less: current portion		$ —	$ (1,315)
Long-term debt, net of current portion		$54,322	$32,304

(in thousands)	March 31, 2024	December 31, 2023

Hercules Loan Agreement

In October 2018, the The Company entered into a Loan and Security Agreement, as most recently amended, with Hercules Capital, Inc., the (“Hercules Loan Agreement, as amended most recently in August 2023, with Hercules. Agreement”). The Hercules Loan Agreement provides for a an aggregate term loan facility of up to $115.0 million, including: (i) $32.5 million outstanding (the “Conversion Balance”) prior to effectiveness under which the Company has borrowed an aggregate of $55.0 million of term loans, representing the most recent amendment in August 2023 (the “Amendment”), (ii) a $22.5 million maximum borrowings allowable as of March 31, 2024. The term loan tranche drawn upon the closing facility allows for $60.0 million of the Amendment, (iii) additional borrowings:

(i) an additional tranche of up to $20.0 million, which will be became available in either one or two drawings following potential on April 26, 2024 upon receipt of U.S. approval of mavorixafor XOLREMDI (mavorixafor) in individuals with WHIM syndrome (“Approval”) syndrome. This tranche is available until the earlier of (A) 45 days following Approval and (B) September 30, 2024 in the case of the first drawing, and until December 15, 2024 in the case of a second drawing, (iv) drawing;

(ii) an additional tranche of $7.5 million, which will be available following achievement of a certain clinical development-related milestone through the earlier of (A) (a) 45 days following achievement of such milestone and (B) (b) December 15, 2024; and (v) (iii) an additional tranche of up to $32.5 million, which will be available subject to approval by Hercules in its sole discretion. under which the Company has borrowed an aggregate of $55.0 million of term loans to date representing the maximum borrowings.

Borrowings under the Hercules Loan Agreement accrue interest at a variable rate equal to the greater of (i) 10.15% or (ii) *The Wall Street Journal* prime rate plus 3.15%. In an event of default and until such event is no longer continuing, the interest rate applicable to borrowings would be increased by 4.0%. Borrowings are repayable in monthly interest-only payments through March 1, 2025 July 1, 2027, and in equal monthly payments of principal and accrued interest from April 1, 2025 (the "Amortization Date") until which is the maturity date of the loans. The Amortization Date may be extended (i) to October 1, 2026, if Approval occurs on or prior to September 30, 2026, and (ii) to the maturity date if an extension pursuant to the foregoing clause (i) has occurred and no event of default occurs. The loans mature on October 1, 2026; *provided, however*, such maturity date will be extended to July 1, 2027 if the Amortization Date is extended pursuant to clause (i) of the foregoing sentence. At the Company's option, the Company may prepay all, but not less than all, of the outstanding borrowings, subject to a prepayment premium of 3% during the first 12 months following January 6, 2023 (the "Original Closing Date"), 2% during the following 12 months month period ending January 5, 2025 and 1% thereafter. In addition, the Hercules Loan Agreement provides for payment of end-of-term fees of $2.1 million plus 3.5% of the aggregate principal amount of future loans drawn, if any, subsequent to the Amendment, payable upon the earlier of maturity or the repayment in full of all obligations under the Hercules Loan Agreement. Borrowings under the Hercules Loan Agreement are

collateralized by substantially all of the Company's personal property and other assets except for its intellectual property (but including rights to payment and proceeds from the sale, licensing or disposition of the intellectual property).

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

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

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- 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 which:
  - (i) the Company maintains Qualified Cash in an aggregate amount equal to at least 75% of loans outstanding under the Amended Loan Agreement or
  - (ii) both (x) (a) the Company maintains a Market Capitalization (as defined in the Hercules Loan Agreement) of at least $450.0 million and (y) (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)	(in thousands)	Three Months Ended September 30,		Nine Months Ended September 30,	

	2023	2022	2023	2022
(in thousands)				
(in thousands)				
	2024			
	2024			
	2024			
Total interest expense				
Total interest expense				
Total interest expense	Total interest expense	$ 1,397	$ 1,018	$ 3,210	$ 2,845
Non-cash interest expense	Non-cash interest expense	$ 237	$ 256	$ 681	$ 653
Non-cash interest expense				
Non-cash interest expense				

The annual effective interest rate of the Hercules Loan Agreement as of September 30, 2023 March 31, 2024 is 13.6%. There were no principal payments due or paid under the Hercules Loan Agreement during the nine three months ended September 30, 2023 March 31, 2024. End-of-term payments of $2.1 million were paid during the nine months ended September 30, 2023.

The Company concluded that the amendments to the Hercules Loan Agreement in January 2023 and August 2023 represented modifications to the debt. Accordingly, fees paid to third parties directly related to the amendments were expensed as incurred and fees paid to Hercules in conjunction with the amendments have been deferred and are being amortized to interest expense over the life of the debt arrangement using the effective interest method.

As of September 30, 2023 March 31, 2024, future principal and accrued end-of-term payments due under the Hercules Loan Agreement were as follows (in thousands):

Year Ending December 31,	Year Ending December 31,	Total	Year Ending December 31,	Total
2023		$ —		
2024	2024	—		
2025	2025	24,720		
2026	2026	30,609		
Long-term debt	Long-term debt	$55,329		
Long-term debt				
Long-term debt				

8. LEASES

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

- ***Vienna Austria Leases—***The Company has an operating lease for approximately 1,200 square meters of laboratory and office space in Vienna, Austria ("Vienna Lease"), which commenced in February 2021 for a term of 7 years. The annual base rent for the Vienna Lease is approximately $285 $282 thousand.

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

- ***Waltham Lease—***The Company leases approximately 6,000 square feet of office space in Waltham, Massachusetts ("Waltham Lease"). The Waltham Lease, as amended, commenced on January 1, 2019, and expires on December 31, 2023. The base rent is approximately $0.3 million annually. In addition to the base rent, the Company is also responsible for its share of operating expenses, electricity and real estate taxes, which costs are not included in the determination of the leases' right-of-use assets or lease liabilities. The Company is subleasing the space to a third party for the duration of the lease. The right-of-use asset is being amortized to rent expense over the five-year term of the lease.

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 nine months ended September 30, 2023 and 2022 were as follows:

(dollars in thousands)	Three Months Ended September 30,		Nine Months Ended September 30,	
Lease Cost	**2023**	**2022**	**2023**	**2022**
Fixed operating lease cost	$ 518	$ 507	$ 1,561	$ 1,561
Total lease expense	$ 518	$ 507	$ 1,561	$ 1,561
Other information				
Operating cash flows from operating leases	$ 345	$ 334	$ 1,037	$ 1,014
Sublease income	$ 49	$ 49	$ 97	$ 147
Weighted-average remaining lease term—operating leases	3.4 years			
Weighted-average discount rate—operating leases	11.5 %			

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

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

Maturities of lease liabilities due under lease agreements that have commenced as of September 30, 2023 March 31, 2024 are as follows (in thousands):

Maturity of lease liabilities	Maturity of lease liabilities	Operating Leases	Maturity of lease liabilities	Operating Leases
2023 (remainder of the year)		$ 403		
2024		1,370		
2024 (remainder of the year)				
2025	2025	1,398		
2026	2026	1,329		
2027	2027	276		
Thereafter		46		
2028				
Total lease payments				
Total lease payments				
Total lease payments	Total lease payments	4,822		
Less: interest	Less: interest	(858)		
Total operating lease liabilities as of September 30, 2023		$ 3,964		

Total operating lease liabilities as of March 31, 2024

9. COMMITMENTS AND CONTINGENCIES

The Company has agreements with clinical research organizations ("CROs") pursuant to which the Company and the CROs are conducting clinical trials. The Company may terminate these agreements by providing notice pursuant to the contractual provisions of such agreements and would incur early termination fees. The Company has agreements with contract manufacturing organizations ("CMOs") for the production of mavorixafor for use in clinical trials. The Company's agreement with the CMO who produces batches of drug substance for use in the Company's clinical and commercial drug supply contains cancellation provisions that would require the Company to pay up to the full contract value upon cancellation. As of September 30, 2023 March 31, 2024, the Company has approximately $1.8 million $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 September 30, 2023 March 31, 2024 or December 31, 2022 December 31, 2023.

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

10. COMMON STOCK AND COMMON STOCK WARRANTS

As of September 30, 2023 March 31, 2024, the Company's Restated Certificate of Incorporation authorized the Company to issue 500 million shares of common stock, par value $0.001 per share. The voting, dividend and liquidation rights of the holders of the Company's common stock are subject to and qualified by the rights, powers and preferences of the holders of any preferred stock that may be issued. Each share of common stock entitles the holder to one vote on all matters submitted to a vote of the Company's stockholders. Common stockholders are entitled to receive dividends, as may be declared by the board of directors, if any. No cash dividends have been declared or paid to date.

Q2 2023 Private Placement

On May 15, 2023, the Company entered into a securities purchase agreement pursuant to which it agreed to issue and sell to several institutional and accredited investors (the "Investors"), in a private placement (the "Q2 2023 Private Placement"),

34,521,046 shares of common stock at a price of $1.52 per share and pre-funded warrants to purchase 8,263,157 shares of common stock at a purchase price of $1.519 per pre-funded warrant (representing the price of $1.52 per share minus the $0.01 per share exercise price of each such prefunded warrant). The pre-funded warrants are exercisable, subject to certain beneficial ownership restrictions, at any time after their original issuance and will not expire. The Q2 2023 Private Placement closed on May 18, 2023. The Company received gross proceeds of $65.0 million, before deducting offering expenses paid by the Company.

Also on May 15, 2023, the Company entered into a registration rights agreement (the "Registration Rights Agreement") with the Investors, pursuant to which the Company agreed to register for resale the common shares issued in the Q2 2023 Private Placement and the issuance of the shares of common stock underlying the pre-funded warrants held by the Investors (the "Registrable Securities"). Under the Registration Rights Agreement, the Company agreed to file a registration statement covering the resale of the Registrable Securities by no later than June 14, 2023. Such registration statement was filed on June 9, 2023 and was declared effective by the SEC on June 20, 2023. The Company has agreed to use commercially reasonable efforts to keep such registration statement effective until the date the shares of common stock sold in the Q2 2023 Private Placement and the shares of common stock underlying the pre-funded warrants covered by such registration statement have been sold or may be resold pursuant to Rule 144 without restriction.

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.

Warrant Exercises

During There were no warrant exercises during the nine three months ended September 30, 2023, 7,475,814 warrants were exercised and the Company received $8.4 million in proceeds. March 31, 2024.

As of September 30, 2023 March 31, 2024, the Company's outstanding warrants and pre-funded warrants to purchase shares of common stock consisted of the following:

Issuance Date	Issuance Date	Number of Shares of Common Stock Issuable	Exercise Price	Expiration Date	Issuance Date	Number of Shares of Common Stock Issuable		Exercise Price		Expiration Date
October 25, 2016	October 25, 2016	5,155	$ 19.78	October 24, 2026	October 25, 2016	5,155	$	$ 19.78	October 24, 2026	October 24, 2026
December 28, 2017	December 28, 2017	115,916	$ 19.78	December 28, 2027	December 28, 2017	115,916	$	$ 19.78	December 28, 2027	December 28, 2027
September 12, 2018	September 12, 2018	20,220	$ 19.78	September 12, 2028	September 12, 2018	20,220	$	$ 19.78	September 12, 2028	September 12, 2028
October 19, 2018	October 19, 2018	20,016	$ 19.78	October 19, 2028	October 19, 2018	20,016	$	$ 19.78	October 19, 2028	October 19, 2028
March 13, 2019	March 13, 2019	5,000	$ 19.78	March 12, 2029	March 13, 2019	5,000	$	$ 19.78	March 12, 2029	March 12, 2029
April 16, 2019	April 16, 2019	3,866,154	$ 13.20	April 15, 2024	April 16, 2019	3,866,154	$	$ 13.20	April 15, 2024	April 15, 2024

November 29, 2019	November 29, 2019	1,250,000	$	12.00	(a)	n/a													
November 29, 2019																			
November 29, 2019												1,250,000		$	12.00			(a)	n/a
March 23, 2021	March 23, 2021	50,000	$	8.70	(b)	n/a				March 23, 2021		50,000	$	$	8.70	(b)		(b)	n/a
November 9, 2021	November 9, 2021	2,008,032	$	4.98	(c)	n/a				November 9, 2021		2,008,032	$	$	4.98	(c)		(c)	n/a
March 3, 2022	March 3, 2022	766,666	$	1.80	(d)	n/a				March 3, 2022		766,666	$	$	1.80	(d)		(d)	n/a
July 6, 2022	July 6, 2022	13,276,279	$	1.095	(e)	n/a				July 6, 2022		13,276,279	$	$	1.095	(e)		(e)	n/a
July 6, 2022	July 6, 2022	44,075,050	$	1.095			July 6, 2027			July 6, 2022		44,075,050	$	$	1.095		July 6, 2027		July 6, 2027
December 9, 2022	December 9, 2022	32,137,448	$	1.50			December 9, 2027			December 9, 2022		32,137,448	$	$	1.50		December 9, 2027		December 9, 2027
December 9, 2022	December 9, 2022	6,800,000	$	1.10	(f)	n/a				December 9, 2022		6,800,000	$	$	1.10	(f)		(f)	n/a
May 19, 2023		8,263,157	$	1.52	(g)	n/a													
		112,659,093																	
May 18, 2023										May 18, 2023		8,263,157	$		1.52			(g)	n/a
	112,659,093																		

(a) In November 2019, the Company received $11.999 per pre-funded warrant, or $21.0 million in aggregate proceeds. Each pre-funded warrant may be exercised for an additional $0.001 per pre-funded warrant. (b) In March 2021, the Company received $8.69 per pre-funded warrant, or $435 thousand in aggregate proceeds. Each pre-funded warrant may be exercised for an additional $0.01 per pre-funded warrant. (c) In November 2021, the Company received $4.97 per pre-funded warrant, or $10.0 million in aggregate proceeds. Each

pre-funded warrant may be exercised for an additional $0.01 per pre-funded warrant. (d) In March 2022, the Company received $1.79 per pre-funded warrant, or $1.4 million in aggregate proceeds. Each pre-funded warrant may be exercised for an additional $0.01 per pre-funded warrant. (e) In July 2022, the Company received $1.094 per pre-funded warrant, or $14.5 million in aggregate proceeds. Each pre-funded warrant may be exercised for an additional $0.001 per pre-funded warrant. (f) In December 2022, the Company received $1.099 per pre-funded warrant, or $7.5 million in aggregate proceeds. (g) In May 2023, the Company received $1.519 per pre-funded warrant, or $12.6 million in aggregate proceeds. Each pre-funded warrant may be exercised for an additional $0.001 per pre-funded warrant.

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11. STOCK-BASED COMPENSATION

Summary of Plans— The Company has the following equity incentive plans:

- The X4 Pharmaceuticals Inc. 2015 Employee, Director and Consultant Equity Incentive Plan, as amended (the "2015 Plan");
- The X4 Pharmaceuticals Inc. 2017 Equity Incentive Plan (the "2017 Plan"); and
- The X4 Pharmaceuticals Inc. Amended and Restated 2019 Inducement Equity Incentive Plan (the "2019 Plan")

The Company also has the following employee stock purchase plan:

- The X4 Pharmaceutical Inc. Amended and Restated 2017 Employee Stock Purchase Plan (the "2017 ESPP")

These plans are administered by the Board of Directors or by a committee of the Board of Directors. The exercise prices, vesting and other restrictions are determined at the discretion of the Board of Directors, or its committee if so delegated, except that the exercise price per share of stock options may not be less than 100% of the fair market value of the share of common stock on the date of grant and the term of the stock option may not be greater than ten years. Incentive stock options granted to employees and restricted stock awards granted to employees, officers, members of the board of directors, advisors, and consultants of the Company typically vest over four years. Non-statutory options granted to employees, officers, members of the board of directors, advisors, and consultants of the Company typically vest over three or four years. Shares that are expired, terminated, surrendered or canceled under the Plans without having been fully exercised will be available for future awards. In addition, shares of common stock that are tendered to the Company by a participant to exercise an award are added to the number of shares of common stock available for the grant of awards.

As of September 30, 2023 March 31, 2024, there are an aggregate of approximately 2.0 4.0 million shares of common stock available for issuance under the Company's equity incentive plans. Approximately 5.0 4.9 million shares of common stock remain available for issuance under the 2017 ESPP.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Risk-free interest rate	4.2 %	3.3 %	4.0 %	2.4 %
Expected term (in years)	6.1	6.1	6.0	6.1
Expected volatility	94.7 %	95.7 %	93.5 %	94.8 %
Expected dividend yield	0 %	0 %	0 %	0 %

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

Stock Options

The following table summarizes the Company's stock option activity for the nine three months ended September 30, 2023 March 31, 2024:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Contractual Term (Years)	Aggregate Intrinsic Value (in thousands)
Outstanding as of December 31, 2022	2,021,480	$ 6.99	6.5	$ 1
Granted	3,648,650	1.31		
Exercised	(2,031)	1.50		
Forfeited and Expired	(99,365)	9.91		
Outstanding as of September 30, 2023	5,568,734	$ 3.22	8.7	$ 164
Exercisable as of September 30, 2023	1,217,001	$ 9.26	5.8	$ 2
Vested and expected to vest as of September 30, 2023	4,102,969	$ 3.86	8.4	$ 108

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

The weighted average grant-date fair value per share of stock options granted during the nine three months ended September 30, 2023 March 31, 2024 and 2022 2023 was $1.02 $0.73 and $1.24, $0.69, respectively.

Restricted Stock Units— The following table summarizes the Company's restricted stock unit activity for the nine three months ended September 30, 2023 March 31, 2024:

	Number of Shares
Unvested as of December 31, 2022 December 31, 2023	1,680,563 3,118,824
Granted	5,064,691 5,882,459
Vested	(926,572) (503,186)
Forfeited	(243,843) (126,511)
Unvested as of September 30, 2023 March 31, 2024	5,574,839 8,371,586

During the nine three months ended September 30, 2023 March 31, 2024, the Company granted performance-based restricted stock units (“PRSUs”) to its employees. The PRSUs vest 50% based on the Company’s achievement of each of two operational milestones conditioned on the grantee’s continued employment with the Company. As of September 30, 2023 March 31, 2024, neither of the two performance criteria had been met. The Company believes that the achievement of these operational milestones is probable and, accordingly, stock-based Stock-based

compensation expense has been recognized for the 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

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the date each 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 September 30, 2023 March 31, 2024, total unrecognized compensation expense related to unvested stock options and restricted stock units was $5.4 million $6.5 million, which is expected to be recognized over a weighted average period of 2.2 2.4 years.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in thousands)	**2023**	**2022**	**2023**	**2022**
Research and development expense	$ 1,259	$ 576	$ 3,210	$ 1,990
Selling, general and administrative expense	1,102	535	2,938	2,102
Total stock-based compensation	$ 2,361	$ 1,111	6,148	4,092

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

Stock Appreciation Rights— On November 7, 2022 February 13, 2024, (the "Grant Date"), the compensation committee of the Board of Directors
approved special retention and recognition grants the grant of stock appreciation rights ("SARs"), pursuant to the 2017 Plan, to the Company's President and Chief Executive Officer, the Company's Chief Financial Officer and Treasurer, and certain other executive officers of the Company. officers. The SARs have a measurement price per SAR equal to $1.80, $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 $1.80 $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.

The calculation of the fair value of the outstanding SARs as of September 30, 2023 includes the closing price of the Company's common stock of $1.09 and the following assumptions on a weighted average basis:

	September 30, 2023
Risk free rate	4.6 %
Expected term (years)	5.11
Expected volatility	97.4 %
Expected dividend yield	— %
Expected forfeiture rate	19 %

The SARs are accounted for as liability awards as settlement will be in the form of cash unless the Board of Directors authorizes settlement in shares of the Company's common stock and such shares are available to be issued from the 2017 Plan. The Company currently intends to settle the SARs in cash if and when exercised. Compensation expense is recorded based the fair value of the SARs, as determined using the Black-Scholes option valuation model, using an accelerated attribution method as the SARs vest. The Company remeasures the fair value of the outstanding SARs each period until settlement and adjusts life-to-date compensation expense to the period end SARs fair value. For the nine months ended September 30, 2023, the Company recognized $2.1 million of compensation expense related to the SARs.

12. INCOME TAXES

The For the three months ended March 31, 2024 and 2023, the Company did not record a U.S. federal or state income tax benefit for its the net operating losses for the nine months ended September 30, 2023 incurred and 2022, research and development credits generated due to the conclusion that uncertainty of realizing a benefit from those items and a full valuation allowance is required against has been applied to the Company's U.S. federal net operating losses and state deferred tax assets. For the nine months ended September 30, 2023 research and 2022, the Company recorded an immaterial development credits as of March 31, 2024 . The income tax provision recorded for the three months ended March 31, 2024 and 2023, primarily related to the Company's Austrian subsidiary and its Austrian subsidiary. Security Corporation subsidiary that holds a portion of its investment portfolio.

13. NET LOSS PER SHARE

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

	Three Months Ended March 31,	Three Months Ended March 31,	Three Months Ended March 31,
(in thousands, except share and per share data)			
(in thousands, except share and per share data)			
(in thousands, except share and per share data)			
Numerator:			
Numerator:			
Numerator:			

		Three Months Ended September 30,		Nine Months Ended September 30,	
(in thousands, except per share data)		**2023**	**2022**	**2023**	**2022**
Numerator:					
Net loss	Net loss	$ (2,305)	$ (21,586)	$ (82,037)	$ (64,763)

Deemed dividend as a result of Class B warrant price reset	—	(287)	—	(2,546)
Net loss attributable to common stockholders	$ (2,305)	$ (21,873)	$ (82,037)	$ (67,309)
Net loss				
Net loss				
Denominator:				
Denominator:				
Denominator:				
Weighted average shares of common stock outstanding—basic and diluted	196,988	83,211	170,751	50,976
Weighted average shares of common stock outstanding—basic and diluted				
Weighted average shares of common stock outstanding—basic and diluted				
Net loss per share attributable to common stockholders—basic and diluted	$ (0.01)	$ (0.26)	$ (0.48)	$ (1.32)
Net loss per share attributable to common stockholders— basic and diluted				
Net loss per share attributable to common stockholders— basic and diluted				

Basic and diluted weighted average shares of common stock outstanding for the three and nine months ended September 30, 2023 March 31, 2024 and September 30, 2022 March 31, 2023 include the weighted average effect of outstanding pre-funded warrants for the purchase of shares of common stock for which the remaining unfunded exercise price is $0.01 or less per share. During the nine months ended September 30, 2022, in accordance with the Company's Class B Warrant agreement, the exercise price of each outstanding Class B Warrant was adjusted to the price of shares of the Company's common stock sold in public or private offerings to the extent such price is lower than the previous Class B warrant price. These price adjustments were accounted for as a deemed dividend that adjusts net loss available to common shareholders for purposes of basic earnings per share. The deemed dividend was calculated using the Black-Scholes pricing model, taking into account historical volatility of the Company's common stock and the estimated remaining life of the outstanding Class B Warrants. The Class B Warrants expired in December 2022.

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 nine

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months ended September 30, 2023 March 31, 2024 and 2022. 2023. All potentially dilutive securities have been excluded from the computation of diluted net loss per share as the effect would be to reduce the net loss per share, and thus they are considered "anti-dilutive." Therefore, the weighted average number of shares of common stock outstanding used to calculate both basic and diluted net loss per share attributable to common stockholders is the same. The Company excluded the following potential shares of common stock, presented based on amounts outstanding at each period end, from the computation of diluted net loss per share attributable to common stockholders for the periods indicated because including them would have had an anti-dilutive effect:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Options to purchase shares of common stock	5,568,734	1,843,673	5,568,734	1,843,673
Unvested restricted stock units	5,574,839	1,397,789	5,574,839	1,397,789
Warrants to purchase shares of common stock (excluding prefunded warrants, which are included in basic shares outstanding)	80,244,959	60,374,393	80,244,959	60,374,393
	91,388,532	63,615,855	91,388,532	63,615,855

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

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

The following information should be read in conjunction with our unaudited condensed consolidated financial statements and the notes thereto included in this Quarterly Report on Form 10-Q and the audited financial information and the notes thereto included in our Annual Report on Form 10-K, which was filed with the Securities and Exchange Commission ("SEC"), on March 21, 2023 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 late clinical-stage biopharmaceutical company discovering, developing, and developing 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.

Our lead clinical candidateOn April 29, 2024, we announced that the U.S. FDA approved our NDA for mavorixafor, which is being marketed under the trade name XOLREMDI™, for use as an oral, once-daily therapy in patients aged 12 years of age and older with WHIM (warts, hypogammaglobulinemia, infections, and myelokathexis) syndrome, to increase the number of circulating mature neutrophils and lymphocytes. WHIM syndrome is a rare combined primary immunodeficiency and chronic neutropenic disorder. Concurrent with the U.S. approval of

XOLREMDI and pursuant to its Rare Pediatric Disease designation, the FDA granted us a Priority Review Voucher that we intend to sell to another drug sponsor.

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

The U.S. approval of XOLREMDI in the WHIM syndrome indication is the first for mavorixafor, which is an orally active bioavailable selective antagonist of chemokine receptor CXCR4, that is being developed as an oral, once-daily therapy. a key regulator of the movement of immune cells throughout the body. Due to its ability to increase the mobilization of mature, functional white blood cells from the bone marrow into the bloodstream, we believe that mavorixafor has the potential to provide therapeutic benefit across a variety of chronic neutropenic immune system disorders and in addition to WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) syndrome, a rare, primary immunodeficiency for which there are no approved therapies. syndrome.

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

This accepted NDA is supported by our successfully completed global, pivotal, Phase 3 clinical trial (4WHIM) evaluating the safety and efficacy of mavorixafor in people with WHIM syndrome. The 4WHIM trial met its primary endpoint and first key secondary endpoint, statistically significantly raising trial participants’ time above threshold for absolute neutrophil counts (“TAT-ANC”) and time above threshold for absolute lymphocyte counts (“TAT-ALC”) versus placebo. Additional data revealed that mavorixafor treatment also resulted in statistically significant reductions in annualized infection rates versus placebo and effected clinically meaningful reductions in the both the severity and duration of infections versus placebo in trial participants. Mavorixafor was well tolerated throughout the 52-week trial.

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

We are also currently advancing mavorixafor for the treatment of people with certain chronic neutropenic disorders following disorders. Following positive results from a Phase 1b clinical trial of a single dose of mavorixafor in people with idiopathic, cyclic, and congenital chronic neutropenia. We neutropenia, we are now conducting a an ongoing Phase 2 clinical trial evaluating the durability, safety, and tolerability of chronic dosing of once-daily oral mavorixafor with or without concurrent treatment with injectable granulocyte colony-stimulating factor (G-CSF) G-CSF in the same patient population. Preliminary results from the trial have shown showed that the first three participants have experienced robust clinically meaningful increases in ANC and have achieved ANC levels in the normal range. An abstract summarizing these data has been accepted for poster presentation at the 65th Annual Meeting of the American Society of Hematology absolute neutrophil count (“ASH” ANC”), taking place from December 9 to December 12, 2023 in San Diego, California. In addition, we . We expect to share further additional data from the Phase 2 trial in the first half of June 2024.

Also in the first half of 2024, we expect We are also planning to initiate a 52-week, global, pivotal, placebo-controlled Phase 3 clinical trial evaluating of mavorixafor in the second quarter of 2024 that aims to evaluate the efficacy, safety, and efficacy tolerability of oral once-daily

mavorixafor (with or without G-CSF) in people aged 12 with congenital or acquired primary autoimmune and older with idiopathic and congenital chronic neutropenia with who are experiencing recurrent and/or without concurrent G-CSF treatment. serious infections.

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

Our Pipeline

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

Macroeconomic Considerations

Unfavorable conditions in the economy in the United States and abroad may negatively affect the growth of our business and our results of operations. For example, macroeconomic events, including the COVID-19 pandemic, rising inflation, the U.S. Federal Reserve raising interest rates, the Russia-Ukraine war and the war in Israel, have led to economic uncertainty globally. The effect of macroeconomic conditions may not be fully reflected in our results of operations until future periods. If, however, economic uncertainty increases or the global economy worsens, our business, financial condition and results of operations may be harmed. For further discussion of the potential impacts of macroeconomic events on our business, financial condition, and operating results, see the section titled “Risk Factors.”

Results of Operations

Comparison of the Three and Nine Months Ended September 30, 2023 March 31, 2024 and 2022 2023

The following table summarizes the results of our operations for the three and nine months ended September 30, 2023 March 31, 2024 and 2022: 2023:

	Three Months Ended March 31,			Three Months Ended March 31,			Three Months Ended March 31,		
	2024			2024			2024		
(in millions)									
(in millions)									
(in millions)									
	Three Months Ended September 30,			Nine Months Ended September 30,					
Operating expenses:									
	2023	2022	Change	2023	2022	Change			
(in thousands)									
Operating expenses:									
Operating expenses:									

Operating expenses:							
Operating expenses:							
Operating expenses:							
Operating expenses:							
Operating expenses:							
Operating expenses:							
Operating expenses:							
Operating expenses:							
Operating expenses:							
Operating expenses:							
Operating expenses:							
Operating expenses:	Operating expenses:						
Research and development	Research and development	$ 19,081	$ 14,110	$ 4,971	$ 56,745	$ 42,044	$ 14,701
Research and development							
Research and development							
Selling, general and administrative	Selling, general and administrative	8,133	6,044	2,089	25,578	20,457	5,121
Gain of sale of non-financial asset		—	—	—	—	(509)	509
Selling, general and administrative							
Selling, general and administrative							
Total operating expenses							
Total operating expenses							
Total operating expenses	Total operating expenses	27,214	20,154	7,060	82,323	61,992	20,331
Loss from operations	Loss from operations	(27,214)	(20,154)	(7,060)	(82,323)	(61,992)	(20,331)
Total other income (expense), net		24,935	(1,445)	26,380	331	(2,757)	3,088
Loss from operations							
Loss from operations							
Total other (expense) income, net							
Total other (expense) income, net							
Total other (expense) income, net							
Loss before provision for income taxes	Loss before provision for income taxes	(2,279)	(21,599)	19,320	(81,992)	(64,749)	(17,243)
Provision for income taxes		26	(13)	39	45	14	31
Loss before provision for income taxes							

Loss before provision for income taxes							
Net loss	Net loss	$ (2,305)	$ (21,586)	$ 19,281	$ (82,037)	$ (64,763)	$ (17,274)
Net loss							
Net loss							

Research and Development Expenses

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

		Three Months Ended March 31,					
		Three Months Ended March 31,					
		Three Months Ended March 31,					
(in millions)							
(in millions)							
Direct research and development expenses by product candidate:							
Direct research and development expenses by product candidate:							
Direct research and development expenses by product candidate:							
Mavorixafor							
Mavorixafor							
Mavorixafor							

		Three Months Ended September 30,			**Nine Months Ended September 30,**		
Unallocated expense							
		2023	**2022**	**Change**	**2023**	**2022**	**Change**
(in thousands							
Direct research and development expenses by product candidate:							
Mavorixafor		$ 10,918	$ 7,217	$ 3,701	$ 34,186	$ 17,894	$ 16,292
X4P-002		(19)	341	(360)	58	2,285	(2,227)
X4P-003		2	3	(1)	2	199	(197)
Unallocated expense							
Unallocated expense	Unallocated expense	8,180	6,549	1,631	22,499	21,666	833
Total research and development expenses	Total research and development expenses	$ 19,081	$ 14,110	$ 4,971	$ 56,745	$ 42,044	$ 14,701
Total research and development expenses							
Total research and development expenses							

Research and development expenses increased $5.0 million and $14.7 million decreased by $2.2 million in the three and nine months ended September 30, 2023 March 31, 2024, respectively, as compared to the corresponding periods same period in the prior year. Research and development expenses in the prior period included a $5.0 million in-license fee related to a development milestone under our Genzyme

agreement. No similar milestone payments were higher incurred in the current three month period as compared to the prior year due to higher regulatory period. Clinical costs, associated with the preparation and submission of our NDA, higher contract manufacturing costs for our drug substance and potential drug product to support the validation of such processes associated with our NDA, and higher third party including third-party costs associated with our pivotal Phase 3 clinical trial of mavorixafor for the treatment of people aged 12 years and older patients with WHIM syndrome. Research syndrome, were lower in the current period due to the completion of this clinical trial in 2023. These decreases in research and development expenses were partially offset by higher in the nine month period as compared to the prior year compensation costs due to the factors discussed above an increase in personnel within our research and due to higher accrued in-license fees, including $5.0 million of accrued fees related to a development milestone under our Genzyme agreement, which we believe is probable of being achieved. functions.

Selling, General and Administrative Expenses

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

During the quarter ended March 31, 2024,we put in place an experienced sales force to support the launch of XOLREMDI. Those costs and other specific costs related to pre-launch activities contributed to the increase in selling, general and administrative expenses during the current period .Selling, general and administrative expenses increased $2.1 million and $5.1 million for the current three and nine month periods, respectively, by approximately $10 million, as compared to the corresponding periods same period in the prior year. These increases were year, primarily driven by by:

- an increase of approximately $5 million in head count and third party compensation costs, as we begin to build out our commercial operations in preparation for the potential approval of our NDA and potential product launch in the first half of 2024, as well as an increase in expense which include higher costs related to our stock appreciation rights (Note 11), which outstanding Stock Appreciation Rights that are measured at fair value at each period. period end until exercised, and an increase in sales and

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

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

Other Income (Expense), Expense, Net

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2023	2022	Change	2023	2022	Change
(in thousands)						
Interest income	$ 1,388	$ 14	$ 1,374	$ 3,137	$ 21	$ 3,116
Interest expense	(1,634)	(1,018)	(616)	(3,891)	(2,849)	(1,042)
Change in fair value of derivative liability	—	—	—	—	511	(511)
Change in fair value of Class C warrant liability	25,164	—	25,164	743	—	743
Other income (expense)	17	(441)	458	342	(440)	782
Total other income (expense), net	$ 24,935	$ (1,445)	$ 26,380	$ 331	$ (2,757)	$ 3,088

	Three Months Ended March 31,

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

Other income (expense), expense, net, for the three months ended September 30, 2023 March 31, 2024 increased significantly approximately $20.0 million as compared to the same periods period in the prior year primarily due to a decrease an increase in the fair value of our Class C warrants, which were issued in the fourth quarter of 2022 and are accounted for as a liability at fair value. We value these our Class C warrants using the Black-Scholes option pricing model, which includes the market value of our common stock as an input. The market price of shares of our common stock decreased increased during the third first quarter of 2023, 2024, which was the primary contributor to the decrease increase in the Class C warrant liability. liability and associated expense. These Class C warrants will continue to be measured at fair value until they are exercised, which will and may continue to generate gains or losses each quarter. Other income (expense), net, also increased in the current period as compared to the same period in the prior year due to an increase in interest income on our money market and marketable security investments due to a general increase in interest rates and an increase in our invested funds. Other income (expense), net, increased for the nine months ended September 30, 2023, as compared to the same period in the prior year as a result of an increase in interest income due to the factors discussed above, partially offset by an increase in interest expense due to an increase in our outstanding borrowings and an increase in the effective interest rate on these borrowings. quarter, until they are exercised.

Provision for Income Taxes

We did not record a U.S. federal or state income tax benefit for our losses for the three and nine months ended September 30, 2023 March 31, 2024 and 2022, 2023, respectively, due to our conclusion that a full valuation allowance is required against our U.S. federal and state deferred tax assets. For the three and nine months ended September 30, 2023 March 31, 2024 and 2022, 2023, we recorded income tax expense related to our Austrian subsidiary and for our Security Corporation subsidiary that holds a portion of our investment portfolio and associated interest income.

Liquidity and Capital Resources

Sources of Liquidity

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

ATM Sales Agreement — We have entered into a Controlled Equity Offering℠ Sales Agreement ("ATM Sales Agreement"), with B. Riley Securities, Inc., Cantor Fitzgerald & Co., and Stifel, Nicolaus & Company, Incorporated 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. For example, most recently in May 2023, we sold shares of common stock and, in lieu of common stock pre-funded warrants to purchase shares of common stock in a private placement ("Q2 2023 Private Placement") offering for gross proceeds of $65.0 million, before offering expenses.

Hercules Loan Agreement —In January 2023, we entered into We have a Second Amended and Restated Loan and Security Agreement, amended from time to time, with Hercules Capital, Inc., as agent and lender, and Hercules Capital Funding IV LLC and Hercules Capital Funding Trust 2022-1, as lenders (collectively, "Hercules"), which agreement amended and restated the Amended and Restated Loan and Security Agreement dated as of June 27, 2019, as subsequently amended from time to time. As of December 31, 2022, we had borrowed $32.5 million under the Second A&R ("Hercules Loan Agreement. On August 2, 2023, we and Hercules entered into an amendment (the "Amendment") to the Second A&R Hercules Loan Agreement, (as amended by the Amendment, the "Hercules Loan Agreement"), which provides for aggregate maximum borrowings of up to $115.0 million. The Hercules Loan Agreement provides for a term loan facility of up to $115.0 million, including under which we have borrowed an aggregate of $55.0 million of term loans to date representing the $32.5 million outstanding prior to the Amendment and a $22.5 million maximum borrowings as of March 31, 2024. The term loan facility allows for $60.0 million in additional borrowings, which includes:

- an additional tranche drawn of up to $20.0 million, which became available on April 26, 2024 in either one or two drawings until June 10, 2024 in the closing case of the Amendment. Additional borrowings are first drawing, and until December 15, 2024 in the case of a second drawing;
- an additional tranche of $7.5 million, which will be available upon the following achievement of operational milestones. Borrowings under a certain clinical development-related milestone through the Hercules Loan Agreement accrues interest at a variable rate equal earlier of (a) 45 days following achievement of such milestone and (b) December 15, 2024; and
- an additional tranche of up to the greater of (i) 10.15% or (ii) 3.15% plus the Wall Street Journal prime rate and are repayable in monthly interest-only payments through March 1, 2025 $32.5 million, and in equal monthly payments of principal and accrued interest from April 1, 2025 until the maturity date of the loans, which is currently October 1, 2026, will be available subject to extension upon our achievement of certain operational milestones. The approval by Hercules Loan Agreement requires that we maintain a minimum level of cash of $20.0 million through January 31, 2025, which amount is subsequently adjusted subject to our achievement of operational milestones. in its sole discretion.

Going Concern— Since our inception, we have incurred significant operating losses and negative cash flows from our operations. We have not yet commercialized any products and we do not expect to generate revenue from sales of any products for several years, if at all. As of September 30, 2023 March 31, 2024, our cash and cash equivalents were $131.6 million $60.5 million, our restricted cash balance was $1.0 million $0.8 million and our investment in marketable securities were $10.1 million $20.4 million. We have a covenant under our Hercules Loan Agreement that currently requires that we maintain a minimum level of cash of $20.0 million through January 31, 2025, subject to subsequent reductions thereafter as further described in Note 7 to our condensed consolidated financial statements. Based on our current cash flow projections, which exclude any benefit from the potential sale of our PRV, no additional borrowings that may become available on Hercules Loan Agreement and with no additional external funding, we believe that we will not be able to maintain the minimum cash required to satisfy this covenant beginning in the first quarter of 2025. In such event, the lenders could require the repayment of all outstanding debt.

We have assessed Management has concluded that substantial doubt exists about our ability to continue as a going concern in accordance with for the requirements one-year period following the issuance of ASC 205-40. We believe that we have adequate financial resources to fund our forecasted operating costs and comply with the minimum cash requirements of our Hercules Loan Agreement for at least one year after the date that these condensed consolidated financial statements were issued on November 9, 2023 for the quarter ended March 31, 2024. Accordingly, the condensed consolidated financial statements have been prepared on a basis that assumes the Company will continue as a going concern, which contemplates the realization of assets and satisfaction of liabilities and commitments in the ordinary course of business.

Based on To finance our current cash flow projections and considering the terms of the Hercules Loan Agreement and with no additional funding, we believe we have sufficient cash, cash equivalents and marketable securities to fund operations, into 2025. However, to finance operations in 2025 and beyond, we will need to raise additional capital, which cannot be assured. Unless and until we reach profitability in the future, we will require additional capital to fund our operations, which could be raised through a combination of equity offerings, debt financings,

other third-party funding, marketing and distribution arrangements and other collaborations and strategic alliances. If we are unable to obtain funding, we could be forced to delay, reduce or eliminate some or all of its 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. operations.

Cash Flows

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

	Nine Months Ended September 30,	
	2023	2022
	(in thousands)	
Net loss	$ (82,037)	$(64,763)
Adjustments to reconcile net loss to net cash used in operating activities	7,570	6,493
Changes in operating assets and liabilities	5,702	261
Net cash used in operating activities	(68,765)	(58,009)
Net cash used in investing activities	(10,050)	(69)
Net cash provided by financing activities	88,419	56,586
Effect of exchange rate changes on cash, cash equivalents and restricted cash	(28)	(468)
Net increase (decrease) in cash, cash equivalents and restricted cash	9,576	(1,960)

	Three months ended March 31,	
	2024	2023
	(in millions)	
Net loss	$ (82,037)	$(64,763)
Adjustments to reconcile net loss to net cash used in operating activities	7,570	6,493
Changes in operating assets and liabilities	5,702	261
Net cash used in operating activities	(68,765)	(58,009)
Net cash used in investing activities	(10,050)	(69)
Net cash provided by financing activities	88,419	56,586
Effect of exchange rate changes on cash, cash equivalents and restricted cash	(28)	(468)
Net increase (decrease) in cash, cash equivalents and restricted cash	9,576	(1,960)

	Net cash used in financing activities		
	Net decrease in cash, cash equivalents and restricted cash		
	Net decrease in cash, cash equivalents and restricted cash		
	Net decrease in cash, cash equivalents and restricted cash		
Cash, cash equivalents and restricted cash, beginning of period	Cash, cash equivalents and restricted cash, beginning of period	123,028	83,108
Cash, cash equivalents and restricted cash, end of period	Cash, cash equivalents and restricted cash, end of period	$132,604	$ 81,148

Operating Activities

During the nine three months ended September 30, 2023 March 31, 2024, net cash used in operating activities was $68.8 million $34 million, primarily resulting from our net loss of $82.0 million $52 million, adjusted for noncash expenses of $7.6 million $16 million and changes in our operating assets and liabilities of $5.7 million $2 million. Non-cash expenses primarily includes a $14 million loss on the change in fair value of our Class C Warrant liability, stock-based compensation expense, non-cash lease expense and non-cash interest expense. Net cash used in operating activities for the nine three months ended September 30, 2022 March 31, 2023 was $58.0 million $27 million, primarily resulting from our net losses of $64.8 million $24 million, adjusted for noncash expenses of $6.5 million and changes in our operating assets and liabilities of $0.3 million $3 million. Net cash used in operating activities increased during the nine three months ended September 30, 2023 March 31, 2024

as compared to the same period in the prior year primarily due to an increase in the payment of our research annual bonuses and development expenses and to a lesser extent increases in our selling, general and administrative pre-commercialization expenses as discussed above. associated with preparations for the U.S. commercial launch of XOLREMDI.

Investing Activities

During the nine three months ended September 30, 2023 March 31, 2024, cash used in investing activities included $10.0 $5 million of net investments in short-term marketable securities. Investing activities in the prior period were not significant.

Financing Activities

There were no cash used in financing activities during the three months ended March 31, 2024. During the nine three months ended September 30, 2023 March 31, 2023, net cash provided by used in financing activities was $88.4 million $2.1 million, consisting primarily of net proceeds from fees paid to Hercules for the Q2 2023 Private Placement, which provided net proceeds amendment and restatement of $60.4 million, and

$22.5 million of new borrowings on our Hercules Loan Agreement. During the nine months ended September 30, 2022, net cash provided by financing activities was $56.6 million, consisting primarily of $60.6 million of net proceeds from two private placement equity offerings that closed during the nine month period and the sale of shares of our common stock to Lincoln Park and through our employee stock purchase plan, partially offset by $1.2 million of end-of-term payments made pursuant to our Hercules Loan Agreement, and fees related to amendments to including the agreement during the period. settlement of a $1 million end-of-term payment.

Funding Requirements

Based on our cash, cash equivalents and marketable securities on hand as of September 30, 2023 March 31, 2024 and the increases to our borrowing capacity noted above and in Note 7 to the accompanying condensed consolidated financial statements, we believe that our cash, cash equivalents and marketable securities will allow us to fund operations into 2025. However, as noted above, based on our current financial projections, which exclude the potential sale of our PRV, additional borrowings under our Hercules Loan Agreement that could become available, and additional c we believe we would be in violation of a minimum cash covenant of the Hercules Loan Agreement in the first quarter of 2025. In order to fund operations and satisfy the minimum cash covenant in the Hercules Loan Agreement, we will be required to raise additional capital, which may be through a combination of equity offerings, debt financings, other third-party funding, marketing and distribution arrangements and other collaborations and strategic alliances.

During 2023 2025 and beyond, assuming no changes to our current operational expectations, we expect our expenses to continue to increase in connection with our ongoing activities, particularly as we advance the current and anticipated clinical trials of our product candidates in development. Because of the numerous risks and uncertainties associated with research, development and commercialization of pharmaceutical product candidates, we are unable to estimate the exact amount of our funding requirements. Our short term and long term funding requirements will depend on and could increase significantly as a result of many factors, including:

- the scope, number, initiation, progress, timing, costs, design, duration, any potential delays, and results of clinical trials and nonclinical studies for our current or future product candidates, particularly our Phase 2 clinical trial of mavorixafor for the treatment of patients individuals with chronic neutropenic disorders;
- the outcome, timing and cost of regulatory reviews, approvals or other actions to meet regulatory requirements established by the FDA and comparable foreign regulatory authorities, including the potential for the FDA or comparable foreign regulatory authorities to require that we perform more studies for our product candidates than those that we currently expect;
- our ability to obtain marketing approval for our product candidates;
- the cost of filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights covering our product and product candidates, including any such patent claims and intellectual property rights that we have licensed from Genzyme pursuant to the terms of our license agreement with Genzyme; and
- our ability to maintain, expand and defend the scope of our intellectual property portfolio, including the cost of defending intellectual property disputes, including patent infringement actions brought by third parties against us or our product or product candidates.

Hercules Loan Agreement

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

Critical Accounting Policies and Significant Judgments and Estimates

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

During the three months ended September 30, 2023 March 31, 2024, there were no material changes to our critical accounting policies as reported for the year ended December 31, 2022 December 31, 2023 as part of our Annual Report. In addition, see Note 2 of these condensed consolidated financial statements under the heading “Recently Adopted Accounting Pronouncements” for new accounting pronouncements or changes to the accounting pronouncements during the three months ended September 30, 2023 March 31, 2024.

Smaller Reporting Company Status

We are a smaller reporting company (“SRC”) as defined by Rule 12b-2 of the Exchange Act and Item 10(f)(1) of Regulation S-K. We may take advantage of certain of the scaled disclosures available to smaller reporting companies for so long as (i) our voting and non-voting common stock held by non-affiliates is less than $250.0 million measured on the last business day of our second fiscal quarter or (ii) our annual revenue is less than $100.0 million during the most recently completed fiscal year and our voting and non-voting common stock held by non-affiliates is less than $700.0 million measured on the last business day of our second fiscal quarter.

Item 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

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

Item 4 CONTROLS AND PROCEDURES

Evaluation of Disclosure Controls and Procedures

Management’s Evaluation of our Disclosure Controls and Procedures

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in the SEC’s rules and forms and accumulated and communicated to our management, including our principal executive officer and principal financial officer, to allow timely decisions regarding required disclosure.

Our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) as of

September 30, 2023, March 31, 2024, and have concluded that, based on such evaluation, our disclosure controls and procedures were effective as of September 30, 2023 March 31, 2024 at the reasonable assurance level. Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Changes in Internal Control Over Financial Reporting

There were no changes in our internal control over financial reporting that occurred during the three months ended September 30, 2023 March 31, 2024 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II: OTHER INFORMATION

Item 1. LEGAL PROCEEDINGS

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

Item 1A. RISK FACTORS

An investment in our securities involves a high degree of risk. You should carefully consider the following information about these risks, together with the other information appearing elsewhere in this Quarterly Report on Form 10-Q, including our unaudited condensed consolidated financial statements and related notes hereto, before deciding to invest in our common stock. The occurrence of any of the

following risks could have a material adverse effect on our business, financial condition, results of operations and future growth prospects or cause our actual results to differ materially from those contained in forward-looking statements we have made in this report and those we may make from time to time. In these circumstances, the market price of our common stock could decline and you may lose all or part of your investment. We cannot assure you that any of the events discussed below will not occur.

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

Risks Related to Our Financial Position and Need for Additional Capital

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

We are a late clinical-stage 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, or become commercially viable. viable, or maintain commercial viability. Since inception, we have incurred significant operating losses. Our net losses were $93.9 million $101.2 million, $88.7 million $93.9 million and $62.1 million $88.7 million for the years ended December 31, 2022 December 31, 2023, 2021 2022 and 2020, 2021, respectively, and were $82.0 million $51.8 million for the nine three months ended September 30, 2023 March 31, 2024. As of September 30, 2023 March 31, 2024, we had an accumulated deficit of $458.8 million $529.7 million. We have funded our operations to date primarily with proceeds from sales of common stock, warrants, and prefunded warrants for the purchase of our preferred stock and our common stock, sales of preferred stock, proceeds from the issuance of convertible debt, and borrowings under loan and security agreements. We have no products one product approved for commercial sale, XOLREMDI, upon which we depend almost entirely on to produce revenue. XOLREMDI, which has been approved for WHIM syndrome in the U.S., faces an unknown market size and growth potential and we have not generated any revenue from product sales to date, and we may never generate product revenue or achieve profitability.

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

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

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

- the scope, number, progress, duration, endpoints, cost, results and timing of clinical trials and nonclinical studies of our current or potential future product candidates, including in particular the scope, progress, duration, endpoints, cost, results and timing for completion of our Phase 2 clinical trial of mavorixafor for the treatment of chronic neutropenic disorders;
- our ability to raise sufficient funds to support the development and potential commercialization of our product candidates;
- the outcomes and timing of regulatory reviews, approvals or other actions;
- 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;
- our ability to manufacture any approved products on commercially reasonable terms;
- our ability to establish a sales and marketing organization or suitable third-party alternatives for any approved product;
- the number and characteristics of product candidates and programs that we pursue;
- hire additional clinical, regulatory and scientific personnel; and
- incur additional legal, accounting and other expenses associated with operating as a public company.

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

We may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors that may alter or delay our plans. For example, As we experienced delays in clinical trial site activation and slower patient enrollment in some of our clinical trials as a result of the COVID-19 pandemic, which delayed our expectations regarding our ability to report data from those trials. Assuming that we complete have completed the development of and obtain obtained marketing approval in the U.S. for any of our product candidates, mavorixafor, we will need to transition from a company with a research and development focus to a company capable of supporting commercial activities. We may encounter unforeseen expenses, difficulties, complications and delays, and may not be successful in such a transition.

We

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

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

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

As of September 30, 2023 March 31, 2024, we have cash and cash equivalents of $131.6 million $60.5 million and marketable securities of $10.1 million $20.4 million. We will require additional capital to sustain our operations, and to carry out our business plans, which may include raising funds through public or private equity or debt financings, third-party funding, marketing and distribution arrangements, as well as other collaborations, strategic alliances and licensing arrangements, or any combination of these approaches. While we have successfully raised

capital in the past, our ability to raise capital in future periods is not assured. We will also require additional capital to satisfy the covenant under our existing debt facility with Hercules Capital, Inc. and certain affiliated entities ("Hercules") that requires that we maintain a minimum level of cash of $20.0 million through January 2025, subsequently and thereafter,

subject to reduction reductions upon the Company's achievement of certain conditions. operational milestones. Based on our current cash flow projections, and assuming no additional funding and no additional inflows of cash from excluding any gross profit related to the potential commercialization sale of our lead product candidate, drug and excluding the potential sale of the priority review voucher that we received upon approval of our, and including additional available borrowings under our existing debt facility but excluding additional borrowings or other sources of external financing, we would fail to maintain the minimum cash required to satisfy this covenant in approximately as soon as the first quarter of 2025. In such event, Hercules the lender could require the repayment of all outstanding debt. Based on the foregoing, we have concluded that substantial doubt exists about our ability to continue as a going concern for a period of at least 12 months from the date of issuance of the financial statements appearing elsewhere in this Quarterly Report. Our financial statements have been prepared assuming that we will continue as a going concern, which contemplates the realization of assets and the settlement of liabilities and commitments in the normal course of business. The financial statements do not include any adjustments relating to the recoverability and classification of recorded asset amounts or the amounts and classification of liabilities that might result from the outcome of the uncertainty described above. See also the risk factor titled "*Our term loan contains restrictions that limit our flexibility in operating our business*" below.

We cannot be certain that additional funding that will be required into 2025 and beyond 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 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 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, including those related to the COVID-19 pandemic, 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 $50.0 million a remaining $47.0 million in the aggregate of shares of our common stock, we do not have any committed external sources of funds and may seek to raise additional capital at any time. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a holder of our common stock. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, declaring dividends or other distributions, acquiring or licensing intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business and may result in liens being placed on additional assets such as intellectual property. For example, our debt facility with Hercules contains a minimum cash financial covenant that we project we would be in violation of in the first quarter of 2025 based on our current cash flow projections, assuming we do not raise additional funding and are not successful in the commercialization of our lead product candidate. funding. If we default on such indebtedness, with Hercules or a future lender, we could be required to pledge additional assets, or the lenders could enforce remedies on the current collateral.

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

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

We have not generated revenues from any product sales since inception and may never become profitable. We may never be able to generate meaningful revenues from sales of XOLREMDI at levels or on timing necessary to support our investment and goals.
To date, we have not generated revenues from any product sales. sales and cannot predict whether when and if we will be able to generate meaningful revenues from sales of XOLREMDI at levels or on timing necessary to support our investment and goals. Our ability to generate revenue and become profitable depends upon our ability to successfully obtain marketing approval and commercialize our product candidates, including mavorixafor, or other product candidates that we may develop, in-license or acquire in the future. Even if we are able to successfully achieve regulatory approval for these product candidates, we are unable to predict the extent of any future losses and do not know when any of these product candidates will generate revenue for us, if at all. Our ability to generate revenue from XOLREMDI , mavorixafor or any of our current or future product candidates also depends on a number of additional factors, including but not limited to our ability to:

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

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

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

Changes in estimates regarding fair value of intangible assets may result in an adverse impact on our results of operations.
We test goodwill for impairment annually or more frequently if changes in circumstances or the occurrence of events suggest impairment exists. Any significant change in market conditions, including a sustained decline in our stock price, that indicate a reduction in carrying value may give rise to impairment in the period that the change becomes known. For example, as of December 31, 2021, our market capitalization, measured as the price of our common stock multiplied by shares of common stock outstanding, declined to below the value of our net assets, including goodwill. As a result of the sustained decline in the market price of our common stock, the fair value of our single reporting unit, measured based on our market capitalization as of December 31, 2021, was lower than its carrying value and we concluded that goodwill was impaired.

Accordingly, we recorded an impairment charge of $9.8 million to reduce the carrying amount of goodwill to $17.4 million as of December 31, 2021. While we determined that goodwill was not impaired based on our quantitative test as of September 30, 2023 March 31, 2024, future declines in the market value of our common stock may result in additional impairment charges being recorded.

Risks Related to Development of Our Product Candidates

We depend almost entirely on the success of our commercial product, XOLREMDI ™, which has been approved for use in patients 12 years of age and older with WHIM syndrome in the U.S., and on our lead product candidate, mavorixafor, which we are developing for the potential treatment of other chronic neutropenic disorders, including WHIM syndrome, and, contingent on a potential strategic partnership, for the treatment of Waldenström's. 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 no products 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 have not yet completed development of any product candidate. We also may not be able to finalize the design or formulation for our other programs. 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 of 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 if any product candidate we develop were to receive though XOLREMDI received marketing approval, or be commercialized for use in combination with other existing therapies, we would continue to be subject to the risks that the FDA or similar regulatory authorities outside of the United States could revoke approval of the therapy used in combination with our product candidate or that safety, efficacy, manufacturing or supply issues could arise with these existing therapies. Combination therapies are commonly used for the treatment of diseases, and we would be subject to similar risks if we develop any of our product candidates for use in combination with other drugs. This could result in our own products being removed from the market or being less successful commercially.

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

If the FDA or similar regulatory authorities outside of the United States do not approve these other drugs or revoke their approval 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 ("MAA") in the European Union from the European Medicines Agency ("EMA"). Commission. Our future NDA submission 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. FDA may convene an advisory committee in connection with review of our NDA, and the outcome of such committee may not favor approval of our NDA. In addition, we may be required by the FDA to conduct additional clinical trials and/or nonclinical studies to support potential approval. Successfully completing clinical trials and obtaining approval of an NDA is a complex, lengthy, expensive and uncertain process, and the FDA, or a comparable foreign regulatory authority, may delay, limit or deny approval of mavorixafor for the treatment of WHIM syndrome or other indications for many reasons, including, among others:

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

The FDA or a comparable foreign regulatory authority may also require more information, including additional nonclinical or clinical data to support approval, which may delay or prevent approval of our commercialization plans, or cause us to abandon the development program. Even if we obtain If our current or future product candidates receive regulatory approval, our 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. For instance, it is possible that mavorixafor could be approved for an indication but fail to be used for treating patients in that indication due to the availability of other available treatments or then-accepted clinical practice.

We depend on license agreements with Genzyme, Beth Israel Deaconess Medical Center, 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 $25.0 million $20.0 million, of which $7.0 million becomes payable 30 days following the FDA's approval of our NDA for the marketing and sale of

mavorixafor in the U.S., contingent upon our achievement of certain late-stage regulatory and sales milestones with respect to licensed products.

- our obligation to pay Genzyme tiered royalties based on net sales of licensed products that we commercialize under the agreement.
- our obligation to pay Genzyme a certain percentage of cash payments received by us or our affiliates in consideration for the grant of a sublicense under the license granted to us by Genzyme.

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

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

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

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

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

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

- the scope of rights granted under the applicable license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property that is not subject to the applicable license agreement;
- our diligence obligations with respect to the use of the licensed technology under the applicable license agreement to develop and commercialize products and technologies, including the level of effort and specific activities that will satisfy

those diligence obligations; and

- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by us and our collaborators.

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

The results of clinical trials may not support our product candidate claims.
Even if our clinical trials are completed as planned, we cannot be certain that their results will support the proposed safety and/or efficacy of our product candidates, that the FDA or foreign government authorities will agree with our conclusions regarding such results, or that the FDA or foreign governmental authorities will not require additional clinical trials. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful and the results of later clinical trials often do not replicate the results of prior clinical trials and preclinical testing. The clinical trial results may fail to demonstrate that our product candidates are safe for humans and effective for the intended indications. This failure could cause us to abandon a product candidate and may delay development of other product candidates. Any delay in, or termination of, our clinical trials will delay or prevent the submission of our marketing applications (NDA and/or MAA) and, ultimately, our ability to obtain approval and commercialize our product candidates and generate product revenues. Information about certain clinical trials, including results (positive or negative) will be made public according to each country's clinical trial register registration policies. Competitors may use this publicly available information to gain knowledge regarding the progress of development programs.

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

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

- delay or failure in reaching agreement with the FDA or a comparable foreign regulatory authority on a trial design that we are able to execute;
- delay or failure in obtaining authorization to commence a trial or inability to comply with conditions imposed by a regulatory authority regarding the scope or design of a clinical trial;
- inability, delay or failure in identifying and maintaining a sufficient number of trial sites, many of which may already be engaged in competing clinical trial programs;
- delay or failure in recruiting and enrolling suitable subjects to participate in a trial;
- delay or failure in having subjects complete a trial or return for post-treatment follow-up;
- clinical sites and investigators deviating from trial protocol, failing to conduct the trial in accordance with regulatory requirements, or dropping out of a trial;
- delay or failure in reaching agreement on acceptable terms with prospective CROs 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 a public health emergency, such as the COVID-19 pandemic, emergencies and unforeseen events such as the wars war in Ukraine and Israel; 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 and expect to continue to experience, a slower enrollment pace. 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 WHIM syndrome and chronic neutropenic disorders, which are rare diseases with limited patient pools from which to draw for clinical trials. The eligibility criteria of our clinical trials will further limit the pool of available trial participants. If we experience difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may be forced to delay, limit or terminate ongoing or planned clinical trials of our product candidates, which would delay our ability to obtain approvals and generate product revenues from any of these product candidates.

If the commercial opportunity for mavorixafor in WHIM syndrome and other chronic neutropenic disorders including WHIM syndrome, is smaller than we anticipate, our potential future revenue from mavorixafor for the treatment of any of the 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 a variety of chronic neutropenic disorders, including WHIM syndrome. We have advanced mavorixafor through a pivotal, Phase 3 clinical trial (the “4WHIM trial) in people with WHIM syndrome, and are currently advancing mavorixafor in a Phase 2 clinical trial in people with certain 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.

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

The results of pre-clinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. Interpretation of results from early, usually smaller, trials that suggest positive trends in some subjects, require caution. Results from later stages of 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 due to the 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 that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Preliminary or top-line data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. Preliminary or top-line data may include, for example, data regarding a small percentage of the patients enrolled in a clinical trial, and such preliminary data should not be viewed as an indication, belief or guarantee that other patients enrolled in such clinical trial will achieve similar results or that the preliminary results from such patients will be maintained. As a result, interim and preliminary data should be viewed with caution until the final data are available. Differences between preliminary or interim data and final data could significantly harm our business prospects and may cause the trading price of our common stock to fluctuate significantly.

Risks Related to the Marketing and Commercialization of Our Product Candidates

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

If we obtain Our approved product and product candidates that receive regulatory approval for a product candidate, it would 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”), or similar strategy, impose significant restrictions on a our product’s indicated uses or marketing, impose ongoing requirements for potentially costly post-approval studies or post-market surveillance. Progress reports are required at quarterly intervals, every six months and at annual intervals depending upon the country, and more frequently if serious adverse events occur.

Our approved product and our product candidates that receive marketing approval will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMP requirements, quality assurance and corresponding maintenance of records and documents and requirements regarding the distribution of samples to physicians and recordkeeping. The marketing approval of our product candidate may be subject to limitations on the indicated uses for which the product may be marketed or to other conditions of approval, or contain requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the drug. The FDA closely regulates the post-approval

marketing and promotion of drugs to ensure that they are marketed only for the approved indications and in accordance with the provisions of the approved labeling.

In addition, manufacturers of drugs and their facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP regulations. In connection with review of our NDA, our manufacturer may be subject to an FDA preapproval inspection, If a regulatory agency discovers previously unknown problems with a 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. For example, any Any regulatory approval that the FDA grants is limited to those indications and patient populations for which a drug is deemed to be safe and effective by the FDA.

While physicians in the United States may choose, and are generally permitted, to prescribe products for uses that are not described in the product's labeling and for uses that differ from those tested in clinical trials and approved by the regulatory authorities, our ability to promote any of our products candidates if approved, will be narrowly 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 has enjoined several companies from engaging in off-label promotion. The government some instances has also required companies to enter into consent decrees corporate integrity agreements or imposed permanent injunctions under which specified promotional conduct is changed or curtailed. If we cannot successfully manage the promotion of our product candidates, if approved, we could become subject to significant liability, which would materially adversely affect our business and financial condition.

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

We have obtained both breakthrough therapy Breakthrough Therapy and Fast Track designations for mavorixafor for the treatment of adult patients with WHIM and we may pursue those designations for other product candidates as well. A breakthrough therapy is defined as a product that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening condition, and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. For product candidates that have been designated as breakthrough therapies, interaction and communication between

the FDA and the sponsor of the trial can help identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. A breakthrough therapy designation affords the possibility of rolling review, enabling the FDA to review portions of our marketing application before submission of a complete application, and possibly, priority review.

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

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

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

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

The FDA has granted rare pediatric disease designation for mavorixafor for the treatment of WHIM syndrome, however, there is no guarantee that FDA approval of mavorixafor for WHIM will result in a priority review voucher.

In 2012, Congress authorized the FDA to award priority review vouchers to sponsors of certain rare pediatric disease product applications. This program is designed to encourage development of new drug and biological products for prevention and treatment of certain rare pediatric diseases. Specifically, under this program, a sponsor who receives an approval for a drug or biologic for a "rare pediatric disease" that meets certain criteria may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product. The sponsor of a rare pediatric disease drug product receiving a priority review voucher may transfer (including by sale) the voucher to another sponsor. The voucher may be further transferred any number of times before the voucher is used, as long as the sponsor making the transfer has not yet submitted the application. The FDA may also revoke any priority review voucher if the rare pediatric disease drug for which the voucher was awarded is not marketed in the U.S. within one year following the date of approval.

The FDA has granted rare pediatric disease designation for mavorixafor for the treatment of WHIM syndrome, however, there is no guarantee that we will be able to obtain a priority review voucher, even if mavorixafor is approved by the FDA. Specifically, FDA may not award the voucher to sponsors of marketing applications unless either (i) the drug has received rare pediatric disease designation as of September 30, 2024 and is then approved by the FDA no later than September 30, 2026; or (ii) Congress reauthorizes the program. Even though we received rare pediatric disease designation by the current statutory deadline of September 30, 2024 we may not receive the voucher if we do not obtain approval by September 2026. Even if legislation is enacted that extends the date by which approval of the rare pediatric disease-designated drug must obtain approval to receive a priority review voucher, we may not obtain approval by that date, and even if we do, we may not obtain a priority review voucher.

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

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

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

Even if we obtain regulatory approval for any of our product candidates that we may develop or acquire in the future, the 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 major operators of hospitals, physicians and patients of the product candidate as a safe and effective treatment, particularly the ability of mavorixafor and our other product candidates to establish themselves as a new standard of care in the treatment paradigm for the indications that we are pursuing;
- the potential and perceived advantages of our products and product candidates over alternative treatments as compared to the their relative costs of the product candidates and alternative treatments; 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 if approved, on hospital or insurance formularies or limitations on coverages that may be available in the early stages of commercialization for newly approved drugs. If any of our product candidates are or any product candidate that is approved but fail 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.

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

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

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

- regulatory authorities may withdraw or limit their approval of such product candidates;

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

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

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

Any product candidate for which we obtain marketing approval will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMP requirements, quality assurance and corresponding maintenance of records and documents and requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the medicine. The FDA closely regulates the post-approval marketing and promotion of drugs to ensure that they are marketed only for the approved indications and in accordance with the provisions of the approved labeling.

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

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions on the labeling, marketing, distribution or use of a product;
- requirements to conduct post-approval clinical trials;
- warning or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenue;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our products;
- product seizure; and
- injunctions or the imposition of civil or criminal penalties.

If, in the future, 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 if and when they are that have been approved.

WeAlthough we have recently begun to build our built a sales or marketing infrastructure, and as an organization we have limited no experience in the sale, sales, marketing or distribution of pharmaceutical products. To achieve commercial success for any our approved product for which we retain sales and marketing responsibilities, we must continue to develop are building a focused sales and marketing organization and/or outsource infrastructure to sell XOLREMDI ™ (mavorixafor) in the U.S. Although our management team has previous experience with such efforts, there can be no assurance that we will be successful in building these functions operations. If we are unable to other third parties. 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 our lead product candidate, mavorixafor for the treatment of potential use in other chronic neutropenic disorders and WHIM syndrome. disorders. We are aware of other companies that are developing CXCR4 inhibitors that are in a similar stage of development as mavorixafor, including BioLineRx, Noxxon, Upsher-Smith, Polyphor and Glycomimetics. To our knowledge, there do not appear to be any competitors with programs in development for WHIM syndrome or chronic neutropenia disorders. With respect to chronic neutropenia, filgrastim injections (human granulocyte colony-stimulating factor (G-CSF)) and two

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

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

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

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

Even if we obtain and maintain approval for our product candidates from the FDA, we may never obtain approval for our product candidates outside of the United States, which would limit our market opportunities and could harm our business.
Approval of a product candidate in the United States by the FDA does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions, and approval by one a foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. If Clinical trials conducted in one country may not be accepted by regulatory approval is obtained, sales of any future product candidates outside of the United States will be subject to foreign regulatory requirements governing clinical trials and marketing approval. authorities in other countries. Even if the FDA grants marketing approval for a product candidate, comparable foreign regulatory authorities also must approve the manufacturing and marketing of the product candidate in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those in the United States, including additional preclinical studies or clinical trials. In many countries outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for any product candidates, if approved, is also subject to approval. Obtaining approval for any future product candidates in the European Union from the European Commission following the opinion of the European Medicines Agency, if we choose to submit a marketing authorization application there, 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.

Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Also, regulatory approval for our product candidates may be withdrawn. If we fail to comply with the regulatory requirements, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed and our business, financial condition, results of operations and prospects could be harmed.

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, 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 as countries and such investment may not be justified from a business standpoint given the COVID-19 pandemic. 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.

Even if we are able to commercialize mavorixafor or any other product candidate Any products that we develop, the product 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 obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the a product, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one XOLREMDI or more future product candidates, even if our product those candidates obtain marketing approval.

Our ability to commercialize mavorixafor XOLREMDI™ or any other future product candidate candidates successfully also will depend depends in part on the extent to which coverage and adequate reimbursement for these products and related treatments will be are available from government health administration authorities, private health insurers and other organizations. Government authorities and other third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the U.S. and E.U. healthcare industries and elsewhere is cost containment.

Government authorities and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that coverage and reimbursement will be available for mavorixafor 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 mavorixafor 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, for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

There may be significant delays in obtaining coverage and reimbursement for newly approved drugs, and coverage may be more limited than the purposes for which the drug is approved by the applicable regulatory authority. Moreover, eligibility for coverage and reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, intellectual property, manufacturing, sale and distribution expenses. Interim reimbursement levels for new drugs, if applicable, may also not be sufficient to cover our costs and may not be made permanent.

Reimbursement rates may vary according to the use of the drug and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs, and may be incorporated into existing payments for other services. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. In the United States, third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. In the European Union, reference pricing systems and other measures may lead to cost containment and reduced prices. Our inability to promptly obtain coverage and adequate reimbursement rates from both government-funded and private payors for XOLREMDI ™ (mavorixafor) or for any future approved products that we develop 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 will be 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.

Although We have an approved, commercialized product, and we do not currently have any drugs on the market, we are and once we begin commercializing our product candidates, we will be subject to additional healthcare statutory and regulatory requirements and enforcement by the federal government and the states and foreign governments in the jurisdictions in which we conduct our business. Healthcare providers and third-party payors will play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our current and future arrangements with third-party payors and customers may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we research, as well as market, sell and distribute XOLREMDI or any products candidates for which we obtain marketing approval. Restrictions under applicable federal and state healthcare approval in the future. These laws and regulations include the following:

- the federal Anti-Kickback Statute prohibits, among other things, persons may restrict or prohibit a wide range of pricing, discounting, marketing and entities from knowingly promotion, structuring and willfully soliciting, offering, receiving or paying remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under a federal healthcare program such as Medicare and Medicaid; a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- the federal false claims laws impose criminal and civil penalties, including civil whistleblower or qui tam actions, against individuals or entities for, among other things, knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government; in addition, the government may assert that a claim including items and services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act;
- the federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA") imposes criminal and civil liability for, among other things, executing a scheme to defraud any healthcare benefit program, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services; similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;
- the federal physician payment transparency requirements, sometimes referred to as the "Sunshine Act" under the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively the "ACA") commission(s), require certain manufacturers of drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid, or the Children's Health Insurance Program to report to the Centers for Medicare & Medicaid Services ("CMS"), information related to payments customer incentive programs and other transfers of value business arrangements generally. Activities subject to physicians, (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain other healthcare professionals (such as physician assistants and nurse practitioners), and teaching hospitals and the ownership and investment interests of physicians and their immediate family members in such manufacturers;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, which also imposes obligations on certain covered entity healthcare providers, health plans, and healthcare clearinghouses as well as their business associates and their subcontractors that perform certain services involving the use or disclosure of individually identifiable health information, and their subcontractors that use, disclose, or otherwise

process individually identifiable health information, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;

- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers;
- some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and may require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers, drug pricing or marketing expenditures;
- state and local laws that require the registration of pharmaceutical sales representatives; and
- state and foreign these laws also govern involve the privacy 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 security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Compliance Requirements."

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to it, we may be subject to significant civil, criminal and administrative penalties, damages, fines, disgorgement, 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 our 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 United States, Medicare covers certain drug purchases by the elderly and eligible disabled people and introduced a reimbursement methodology based on average sales prices for physician-administered drugs. In addition, Medicare may limit the number of drugs that will be covered in any therapeutic class. Ongoing cost reduction initiatives and future laws could decrease the coverage and price that we will receive for any approved products. While Medicare beneficiaries are limited to most elderly and certain disabled individual, private payors often follow Medicare coverage policy and payment limitations in setting their own payment rates.

In March 2010, the ACA became law. The ACA is a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for the healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. Among the provisions of the ACA of importance to our product candidates are the following:

- an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic products;
- an increase approval in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;

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

There have been challenges to certain aspects of the ACA. In addition, other legislative changes have been proposed and adopted since the ACA was enacted. For example, on June 17, 2021, the U.S. Supreme Court dismissed a challenge on procedural grounds that argued the ACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress. Prior to the U.S. Supreme Court ruling, on January 28, 2021, President Biden issued an executive order to initiate a special enrollment period for purposes of obtaining health insurance coverage through the ACA marketplace. The executive order also instructed certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the ACA. In addition, on August 16, 2022, President Biden signed the Inflation Reduction Act of 2022 into law, which among other things, extends enhanced subsidies for individuals purchasing health insurance coverage in ACA marketplaces through plan year 2025. The IRA also eliminates the "donut hole" under the Medicare Part D program beginning in 2025 by significantly lowering the beneficiary maximum out-of-pocket cost and creating a new manufacturer discount program. It is possible that the ACA will be subject to judicial or Congressional challenges in the future. It is unclear how any such challenges and the healthcare reform measures of the Biden administration will impact the ACA and our business.

There has also been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several Presidential executive orders, Congressional inquiries and proposed bills designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug products. For example, in July 2021, the Biden administration released an executive order, "Promoting Competition in the American Economy," with multiple provisions aimed at prescription drugs. In response to Biden's executive order, on September 9, 2021, the U.S. Department of Health and Human Services ("HHS") released a Comprehensive Plan for Addressing High Drug Prices that outlines principles for drug pricing reform and sets out a variety of potential legislative policies that Congress could pursue as well as potential administrative actions HHS can take to advance these principles. In addition, the IRA, among other things, (i) directs HHS to negotiate the price of certain high-expenditure, single-source drugs and biologics covered under Medicare, and subject drug manufacturers to civil monetary penalties and a potential excise tax by offering a price that is not equal to or less than the negotiated "maximum fair price" for such drugs and biologics under the law, and (ii) imposes rebates with respect to certain drugs and biologics covered under Medicare Part B or Medicare Part D to penalize price increases that outpace inflation. The IRA permits HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years. HHS has and will continue to issue and update guidance as these programs are implemented. These provisions will take effect progressively starting in fiscal year 2023, On August 29, 2023, HHS announced the list of the first ten drugs that will be subject to price negotiations, although the Medicare drug price negotiations program is currently subject to legal challenges. It is currently unclear how the IRA will be implemented but is likely to have a significant impact on the pharmaceutical industry. Further, in response to the Biden administration's October 2022 executive order, on February 14, 2023, HHS released a report outlining three new models for testing by the CMS Innovation Center which will be evaluated on their ability to lower the cost of drugs, promote accessibility, and improve quality of care. It is unclear whether the models will be utilized in any health reform measures in the future. At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

We cannot predict what healthcare reform initiatives may be adopted in the future. However, we expect that the ACA, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we will may receive for any approved product. These new laws may result in additional reductions in Medicare and other healthcare funding. Any reduction in payments from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our products.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals, if any, of our product candidates operations may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval for our future product candidates, as well as subject us to more stringent product labeling and post-marketing conditions and other requirements.

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

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

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

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

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

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

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

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

public company, future government shutdowns could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

Risks Related to Our Dependence on Third Parties

We have no experience manufacturing our product or product candidates on a large clinical or commercial scale and have no manufacturing facility. We are currently dependent on a single third party manufacturer for the manufacture of mavorixafor, 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 manufacturer 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 and any products that we may eventually commercialize be manufactured according to cGMP and similar foreign standards. Drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and some state agencies, and are subject to periodic unannounced inspections for compliance with cGMP requirements. Changes to the manufacturing process are strictly regulated and often require prior FDA or other regulatory authority approval before being implemented. FDA requirements also require investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon us and any third-party manufacturers that we may decide to use. Accordingly, the manufacturers must continue to expend time, money and effort in the areas of production and quality control to maintain cGMP compliance. Any failure by our third-party manufacturers to comply with cGMP or failure to scale up manufacturing processes, including any failure to deliver sufficient quantities of product candidates or products if they are approved in a timely manner, could lead to a delay in, or failure to obtain, regulatory approval of any of our product candidates. In addition, such failure could be the basis for the FDA to issue a warning letter, withdraw approvals for product candidates previously granted to us, or take other regulatory or legal action, including recall or seizure, total or partial suspension of production, suspension of ongoing clinical trials, refusal to approve pending applications or supplemental applications, detention of product, refusal to permit the import or export of products, injunction, or imposing civil and criminal penalties.

Our current manufacturer 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 no 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. 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 and conduct bioresearch monitoring audits in connection with review of NDAs. 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 issue a Complete Response Letter and require us to perform additional clinical trials before approving our marketing applications.

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

Disruptions in our supply chain could delay the commercial launch of our product candidates. or product candidates, if approved.
Any significant disruption in our supplier relationships could harm our business. We currently rely on a single source supplier of mavorixafor, as well a single supplier for the finished product capsules for mavorixafor. If either of these single source suppliers suffers a major natural or man-made disaster at its manufacturing facility, we would not be able to manufacture mavorixafor on a commercial scale until a qualified alternative supplier is identified. Although alternative sources of supply exist, the number of third party suppliers with the necessary manufacturing and regulatory expertise and facilities is limited, and it could be expensive and take a significant amount of time to arrange for alternative suppliers. Any significant delay in the supply of a product or product candidate or its key materials for an ongoing clinical study could considerably delay completion of our clinical studies, product testing and potential regulatory approval of our product candidates. If we or our manufacturers are unable to purchase these key materials after regulatory approval has been obtained for 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. For example, recent judicial decisions in the U.S. raised questions regarding the award of patent term adjustment (PTA) for patents in families where related patents have issued without PTA. Thus, it cannot be said with certainty how PTA will be viewed in the future and whether patent expiration dates may be impacted. 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, the a new unitary patent system that came into effect in June 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. For example, our former interim Chief Medical Officer transitioned back to our Board of Directors and we hired a permanent Chief Medical Officer in August 2023. 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 September 30, 2023 March 31, 2024, we had 86 116 full-time employees. As our development and commercialization plans and strategies develop, or as a result of any future acquisitions, we will need additional managerial, operational, development, sales, marketing, financial and other resources. Our management, personnel and systems currently in place will not be adequate to support this future growth. Future growth would impose significant added responsibilities on our employees, including:

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

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

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

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

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

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

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

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

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

Our ability to use our net operating losses to offset future taxable income may be subject to certain limitations.
Our net operating loss (“NOL”) carryforwards could expire unused and be unavailable to offset future tax liabilities because of their limited duration or because of restrictions under U.S. tax law. As of December 31, 2022 December 31, 2023, we had U.S. federal and state NOLs of $342.9 million $400.0 million and $336.5 million $389.0 million, respectively. Our NOLs generated in tax years ending on or prior to December 31, 2017 are only permitted to be carried forward for 20 years under applicable U.S. tax law. Under the Tax Act, as modified by the CARES Act, our federal NOLs generated in tax years ending after December 31, 2017 may be carried forward indefinitely, but the deductibility of federal NOLs, particularly for tax years beginning after December 31, 2020, may be limited. It is uncertain if and to what extent various states will conform to the Tax Act and the CARES Act.

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

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

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

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

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

Our business could be adversely affected by economic downturns, inflation, increases in interest rates, natural disasters, public health crises, such as the COVID-19 pandemic, political crises, geopolitical events, such as the wars war in Ukraine and Israel, 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 wars war in Ukraine and Israel, or in Gaza, it may make any necessary debt or equity financing more difficult to obtain in a timely manner or on favorable terms, more costly or more dilutive. Increased inflation rates can adversely affect us by increasing our costs, including labor and employee benefit costs.

Risks Related to Ownership of Our Common Stock

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

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

cause the market price of our common stock to fluctuate include:

- our ability or the ability of our collaborators to develop product candidates and conduct clinical trials that demonstrate such product candidates are safe and effective;
- our ability or the ability of our collaborators to obtain regulatory approvals for product candidates, and delays or failures to obtain such approvals;
- failure of any our product candidates to demonstrate safety and efficacy, receive regulatory approval and achieve commercial success;
- failure to maintain our existing third-party license, manufacturing and supply agreements;

    - failure by us or our licensors to prosecute, maintain or enforce our intellectual property rights;
- changes in laws or regulations applicable to our current or future product candidates;
- any inability to obtain adequate supply of product candidates or the inability to do so at acceptable prices;

- adverse decisions by regulatory authorities;
- introduction of new or competing products by our competitors;
- failure to meet or exceed financial and development projections that we may provide to the public;
- the perception of the pharmaceutical industry by the public, legislatures, regulators and the investment community;
- announcements of significant acquisitions, strategic collaborations, joint ventures or capital commitments by us or our competitors;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain intellectual property protection for our technologies;
- additions or departures of key personnel;
- significant lawsuits, including intellectual property or stockholder litigation;
- announcements by us of material developments in our business, financial condition and/or operations;
- if securities or industry analysts do not publish research or reports about us, or if they issue an adverse or misleading opinions regarding our business and stock;
- changes in the market valuations of similar companies;
- general macroeconomic, political and market conditions and overall fluctuations in the financial markets in the United States and abroad;
- sales of our common stock or our stockholders in the future;
- trading volume of our common stock;
- adverse publicity relating to our markets generally, including with respect to other products and potential products in such markets;
- changes in the structure of health care payment systems;
- period-to-period fluctuations in our financial results; and
- the other factors described in this “Risk Factors” section and elsewhere in this Quarterly 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, including for example in connection with the COVID-19 pandemic, 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, or developments relating to the COVID-19 pandemic, 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.

Our common stock may be delisted from The Nasdaq Capital Market which could negatively impact the price of our common stock, liquidity and our ability to access the capital markets.

Our common stock is currently listed on The Nasdaq Capital Market under the symbol “XFOR.” The listing standards of The Nasdaq Capital Market provide that a company, in order to qualify for continued listing, must maintain a minimum stock price of $1.00 and satisfy standards

relative to minimum stockholders' equity, minimum market value of publicly held shares and various additional requirements. If Nasdaq delists our securities from trading on its exchange for failure to meet the listing standards, we and our stockholders could face significant negative consequences including:

- limited availability of market quotations for our securities;
- a determination that the common stock is a "penny stock" which would require brokers trading in the common stock to adhere to more stringent rules, possibly resulting in a reduced level of trading activity in the secondary trading market for shares of common stock;
- a limited amount of analyst coverage, if any; and
- a decreased ability to issue additional securities or obtain additional financing in the future.

Delisting from The Nasdaq Capital Market could also result in other negative consequences, including the potential loss of confidence by suppliers, customers and employees, the loss of institutional investor interest and fewer business development opportunities.

In particular, our share price may continue to decline for a number of reasons, including many that are beyond our control. See the risk factor captioned "Our stock price has been and is likely to continue to be volatile and fluctuate substantially"

"Penny stock" rules may make buying or selling our securities difficult which may make our stock less liquid and make it harder for investors to buy and sell our securities.
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 may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain, if any, for the foreseeable future. We are prohibited from declaring or paying any cash dividends under our existing loan and security agreement with Hercules.

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

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

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

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

Provisions in our corporate charter documents and under Delaware law could make an acquisition of our company, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

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

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

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

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

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

Our certificate of incorporation provides that the Court of Chancery of the State of Delaware is the exclusive forum for any derivative action or proceeding brought on the Company's behalf, any action asserting a breach of fiduciary duty owed by our directors, officers, other employees or stockholders to the Company or our stockholders, any action asserting a claim against the Company arising pursuant to the Delaware General Corporation Law or as to which the Delaware General Corporation Law confers jurisdiction on the Court of Chancery of the State of

Delaware, or any action asserting a claim arising pursuant to our certificate of incorporation or by-laws or governed by the internal affairs doctrine. This provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with the Company or our directors, officers, employees or stockholders, which may discourage such lawsuits against the Company and our directors, officers, employees or stockholders.

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

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

None.

Item 3. DEFAULTS UPON SENIOR SECURITIES

None.

Item 4. MINE SAFETY DISCLOSURES

Not applicable.

Item 5. OTHER INFORMATION

Rule 10b5-1 Trading Plans

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

Item 6. EXHIBITS

		Incorporated by Reference to:			
Exhibit No.	Exhibit Description	Form	Exhibit No.	Filing Date	File No.
3.1	Restated Certificate of Incorporation, as amended, as of September 1, 2022.	8-K	3.1	09/01/2022	001-38295
3.2	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*+	First Amendment to Second Amended and Restated Loan and Security Agreement, dated as of August 2, 2023, by and among X4 Pharmaceuticals, Inc., X4 Therapeutics, Inc., Hercules Capital, Inc. and Hercules Capital Funding IV LLC and Hercules Capital Funding Trust 2022-1.				
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*	XBRL Taxonomy Extension Presentation Linkbase Document
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101)

		Incorporated by Reference to:			
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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.

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

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: November 9, 2023 May 7, 2024

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

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

Date: November 9, 2023 May 7, 2024

By: /s/ Adam S. Mostafa

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

Exhibit 10.1

Certain schedules and exhibits have been omitted from this Exhibit pursuant to Item 601(a)(5) of Regulation S-K. X4 Pharmaceuticals, Inc. will furnish a copy of any omitted schedule or exhibit to the U.S. Securities and Exchange Commission or its staff upon request.

FIRST AMENDMENT TO SECOND AMENDED AND RESTATED LOAN AND SECURITY AGREEMENT

THIS FIRST AMENDMENT TO SECOND AMENDED AND RESTATED LOAN AND SECURITY AGREEMENT (this "Amendment"), dated as of August 2, 2023, is entered into by and among X4 PHARMACEUTICALS, INC., a Delaware corporation (formerly known as ARSANIS, INC.) ("X4 Pharmaceuticals"), X4 THERAPEUTICS, INC., a Delaware corporation (formerly known as X4 PHARMACEUTICALS, INC.) ("X4 Therapeutics"; togaether with X4 Pharmaceuticals, collectively, "Borrower"), the several banks and other financial institutions or entities from time to time parties to the Loan Agreement (collectively, referred to as the "Lender"), and HERCULES CAPITAL, INC., a Maryland corporation, in its capacity as administrative agent and collateral agent for itself and the Lender (together with its successors and assigns, in such capacity, the "Agent").

1. Borrower, Lender and Agent are parties to that certain Second Amended and Restated Loan and Security Agreement, dated as of January 6, 2023 (as amended, restated, supplemented or otherwise modified from time to time prior to the

date of this Amendment, the "Loan Agreement"). Lender has extended credit to Borrower for the purposes permitted in the Loan Agreement.

2. Borrower, Agent and Lender have agreed to certain amendments to the Loan Agreement upon the terms and conditions more fully set forth herein.

SECTION 1 Definitions; Interpretation.

(a) **Terms Defined in Loan Agreement**. All capitalized terms used in this Amendment (including in the recitals hereof) and not otherwise defined herein shall have the meanings assigned to them in the Loan Agreement (as amended by this Amendment).

(b) **Rules of Construction**. The rules of construction in the final paragraph of Section 1.2 of the Loan Agreement shall be applicable to this Amendment and are incorporated herein by this reference.

SECTION 2 Amendment to the Loan Agreement. Upon the occurrence of the First Amendment Effective Date, the Loan Agreement is hereby amended by (i) deleting the stricken text (indicated textually in the same manner as the following example: ~~stricken text~~), and (ii) adding the double underlined text (indicated textually in the same manner as the following example: double-underlined text) as set forth in the amended Loan Agreement attached hereto as Exhibit A.

SECTION 3 Facility Fee. On the date hereof, Borrowers shall pay to Agent a facility fee (the "First Amendment Facility Fee") equal to Two Hundred Fifty Thousand Dollars ($250,000), which shall be nonrefundable and in immediately available funds in U.S. dollars, without setoff, recoupment or counterclaim, and free and clear of and without deduction for any and all present or future taxes, levies, imposts, duties, deductions, withholdings, assessments, fees or other charges imposed by any governmental authority (including any interest, additions to tax or penalties applicable thereto) except as otherwise required by law.

SECTION 4 Conditions of Effectiveness. The effectiveness of this Amendment (the "First Amendment Effective Date") shall be subject to Agent's receipt of the following documents, in form and substance satisfactory to Agent, or, as applicable, the following conditions being met:

(a) this Amendment, executed by Agent, Lender and Borrower;

(b) Borrower shall have paid (i) the First Amendment Facility Fee, (ii) all invoiced costs and expenses then due in accordance with Section 8(d), and (iii) all other fees, costs and expenses, if any, due and payable as of the date hereof under the Loan Agreement;

(c) Borrower shall have submitted an Advance Request for the Tranche 2 Advance;

(d) copy of resolutions of Borrower's Board of Directors, certified by an officer of Borrower, evidencing (i) approval of this Amendment, (ii) authorizing a specified person or persons to execute this Amendment on its behalf, and (iii) acknowledging that the Board of Directors are acting for a proper purpose and that this Amendment is in the best interests of Borrower and for its commercial benefit;

(e) a legal opinion of Borrower's counsel in form and substance reasonably acceptable to Agent;

(f) certified copies, dated as of a recent date, of searches for financing statements filed in the central filing office of the State of Delaware, accompanied by written evidence (including any UCC termination statements) that the Liens on any Collateral indicated in any such financing statements either constitute Permitted Liens or have been or, in connection with the Tranche 2 Advance, will be terminated or released;

(g) on the First Amendment Effective Date, immediately after giving effect to the amendments of the Loan Agreement contemplated hereby:

(i)the representations and warranties contained in Section 5 shall be true and correct in all material respects on and as of the First Amendment Effective Date as though made on and as of such date; *provided*, *however*, that such materiality qualifier shall not be applicable to any representations and warranties that already are qualified or modified by materiality in the text thereof; *provided, further,* that to the extent such representations and warranties by their terms expressly relate only to a prior date such representations and warranties shall be true and correct as of such prior date; and

(ii)there exist no Defaults or Events of Default; and

(h) such other documents or evidence as Agent may reasonably request to effectuate the terms of this Amendment.

SECTION 5 Representations and Warranties. To induce Agent and Lender to enter into this Amendment, Borrower hereby confirms, as of the date hereof, (a) that the representations and warranties made by it in Section 5 of the Loan Agreement and in the other Loan Documents are true and correct in all material respects; *provided*, *however*, that such materiality qualifier shall not be applicable to any representations and warranties that already are qualified or modified by materiality in the text thereof; *provided, further,* that to the extent such representations and warranties by their terms expressly relate only to a prior date such representations and warranties shall be true and correct as of such prior date; (b) that no Event of Default has occurred and is continuing; (c) that there has not been and there does not exist a Material Adverse Effect; (d) Lender has and shall continue to have valid, enforceable and perfected first-priority liens, subject only to Permitted Liens, on and security interests in the Collateral and all other collateral heretofore granted by Borrower to Lender, pursuant to the Loan Documents or otherwise granted to or held by Lender; (e) the agreements and obligations of Borrower contained in the Loan Documents and in this Amendment constitute the legal, valid and binding obligations of Borrower, enforceable against Borrower in accordance with their respective terms, except as the enforceability thereof may be limited by bankruptcy, insolvency or other similar laws of general application affecting the enforcement of creditors' rights or by the application of general principles of equity; and (f) the execution, delivery and performance of this Amendment by Borrower will not violate (1) any organizational document of Borrower, (2) any material law, rule, regulation or order to which Borrower is subject, or (3) except as described on Schedule 5.3 to the Loan Agreement, any material contractual obligation of Borrower and will not result in, or require, the creation or imposition of any lien, claim or encumbrance of any kind on any of its properties or revenues, other than Permitted Liens and the Liens created by the Loan Agreement and the other Loan Documents. For the purposes of this Section 5, each

reference in Section 5 of the Loan Agreement to “this Agreement”, and the words “hereof”, “herein”, “hereunder”, or words of like import in such Section, shall mean and be a reference to the Loan Agreement as amended by this Amendment.

SECTION 6 **Release.**In consideration of the agreements of Agent and each Lender contained herein and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, Borrower, on behalf of itself and its successors, assigns, and other legal representatives, hereby to the extent possible under applicable law fully, absolutely, unconditionally and irrevocably releases, remises and forever discharges Agent and each Lender, and its successors and assigns, and its present and former shareholders, affiliates, subsidiaries, divisions, predecessors, directors, officers, attorneys, employees, agents and other representatives (Agent, Lender and all such other persons being hereinafter referred to collectively as the “Releasees” and individually as a “Releasee”), of and from all demands, actions, causes of action, suits, covenants, contracts, controversies, agreements, promises, sums of money, accounts, bills, reckonings, damages and any and all other claims, counterclaims, defenses, rights of set-off, demands and liabilities whatsoever of every name and nature, known or unknown, suspected or unsuspected, both at law and in equity, which Borrower, or any of its successors, assigns, or other legal representatives may now or hereafter own, hold, have or claim to have against the Releasees or any of them for, upon, or by reason of any circumstance, action, cause or thing whatsoever which arises at any time on or prior to the day and date of this Amendment, for or on account of, or in relation to, or in any way in connection with the Loan Agreement, or any of the other Loan Documents or transactions thereunder or related thereto. Borrower understands, acknowledges and agrees that the release set forth above may be pleaded as a full and complete defense and may be used as a basis for an injunction against any action, suit or other proceeding which may be instituted, prosecuted or attempted in breach of the provisions of such release. Borrower agrees that no fact, event, circumstance, evidence or transaction which could now be asserted or which may hereafter be discovered shall affect in any manner the final, absolute and unconditional nature of the release set forth above.

SECTION 7 **Tranche 2 Advance**. Lender shall fund the Tranche 2 Advance on the First Amendment Effective Date in accordance with the Advance Request delivered pursuant to Section 4(c).

SECTION 8 **Miscellaneous**.

(a) **Loan Documents Otherwise Not Affected; Reaffirmation; No Novation**.

(i)Except as expressly amended pursuant hereto or referenced herein, the Loan Agreement and the other Loan Documents shall remain unchanged and in full force and effect and are hereby ratified and confirmed in all respects. The Lender’s and Agent’s execution and delivery of, or acceptance of, this Amendment shall not be deemed to create a course of dealing or otherwise create any express or implied duty by any of them to provide any other or further amendments, consents or waivers in the future.

(ii)Borrower hereby expressly (1) reaffirms, ratifies and confirms its Secured Obligations under the Loan Agreement and the other Loan Documents, (2) reaffirms, ratifies and confirms the grant of security under Section 3 of the Loan Agreement, (3) reaffirms that such grant of security in the Collateral secures all Secured Obligations under the Loan Agreement, including without limitation any Term Loan Advances funded on or after the First Amendment Effective Date, as of the date hereof, and with

effect from (and including) the First Amendment Effective Date, such grant of security in the Collateral: (x) remains in full force and effect notwithstanding the amendments expressly referenced herein; and (y) secures all Secured Obligations under the Loan Agreement, as amended by this Amendment, and the other Loan Documents, (4) agrees that this Amendment shall be a "Loan Document" under the Loan Agreement, and (5) agrees that the Loan Agreement and each other Loan Document shall remain in full force and effect following any action contemplated in connection herewith.

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(iii)This Amendment is not a novation and the terms and conditions of this Amendment shall be in addition to and supplemental to all terms and conditions set forth in the Loan Documents. Nothing in this Amendment is intended, or shall be construed, to constitute an accord and satisfaction of Borrower's Secured Obligations under or in connection with the Loan Agreement and any other Loan Document or to modify, affect or impair the perfection or continuity of Agent's security interest in, (on behalf of itself and the Lender) security titles to or other liens on any Collateral for the Secured Obligations.

(b) **Conditions**. For purposes of determining compliance with the conditions specified in Section 4, each Lender that has signed this Amendment shall be deemed to have consented to, approved or accepted or to be satisfied with, each document or other matter required thereunder to be consented to or approved by or acceptable or satisfactory to the Lender unless Agent shall have received notice from such Lender prior to the date hereof specifying its objection thereto.

(c) **No Reliance**. Borrower hereby acknowledges and confirms to Agent and Lender that Borrower is executing this Amendment on the basis of its own investigation and for its own reasons without reliance upon any agreement, representation, understanding or communication by or on behalf of any other Person.

(d) **Costs and Expenses**. Borrower agrees to pay to Agent on the date hereof the out-of-pocket costs and expenses of Agent and each Lender party hereto, and the fees and disbursements of counsel to Agent and each Lender party hereto (including allocated costs of internal counsel) in connection with the negotiation, preparation, execution and delivery of this Amendment and any other documents to be delivered in connection herewith on the date hereof.

(e) **Binding Effect**. This Amendment binds and is for the benefit of the successors and permitted assigns of each party.

(f) **Governing Law.** THIS AMENDMENT AND THE OTHER LOAN DOCUMENTS SHALL BE GOVERNED BY, AND CONSTRUED AND ENFORCED IN ACCORDANCE WITH, THE LAWS OF THE STATE OF CALIFORNIA, EXCLUDING CONFLICT OF LAWS PRINCIPLES THAT WOULD CAUSE THE APPLICATION OF LAWS OF ANY OTHER JURISDICTION.

(g) **Complete Agreement; Amendments**. This Amendment and the Loan Documents represent the entire agreement about this subject matter and supersede prior negotiations or agreements with respect to such subject matter. All prior

agreements, understandings, representations, warranties, and negotiations between the parties about the subject matter of this Amendment and the Loan Documents merge into this Amendment and the Loan Documents.

(h) **Severability of Provisions.** Each provision of this Amendment is severable from every other provision in determining the enforceability of any provision.

(i) **Counterparts**. This Amendment may be executed in any number of counterparts and by different parties on separate counterparts, each of which, when executed and delivered, is an original, and all taken together, constitute one Amendment. Delivery of an executed counterpart of a signature page of this Amendment by facsimile, portable document format (.pdf) or other electronic transmission will be as effective as delivery of a manually executed counterpart hereof.

(j) **Electronic Execution of Certain Other Documents**. The words "execution," "execute", "signed," "signature," and words of like import in or related to any document to be signed in connection with this Amendment and the transactions contemplated hereby (including without limitation assignments, assumptions, amendments, waivers and consents) shall be deemed to include electronic signatures, the electronic matching of assignment terms and contract formations on electronic platforms

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approved by the Agent, or the keeping of records in electronic form, each of which shall be of the same legal effect, validity or enforceability as a manually executed signature or the use of a paper-based recordkeeping system, as the case may be, to the extent and as provided for in any applicable law, including the Federal Electronic Signatures in Global and National Commerce Act, the California Uniform Electronic Transactions Act, or any other similar state laws based on the Uniform Electronic Transactions Act.

[REMAINDER OF PAGE INTENTIONALLY LEFT BLANK]

IN WITNESS WHEREOF, the parties hereto have duly executed this Amendment, as of the date first above written.

BORROWER:

X4 PHARMACEUTICALS, INC. (F/K/A ARSANIS, INC.)

Signature: /s/ Adam Mostafa

Print Name: Adam Mostafa

Title: Chief Financial Officer

X4 THERAPEUTICS, INC. (F/K/A X4 PHARMACEUTICALS, INC.)

Signature: /s/ Adam Mostafa

Print Name: Adam Mostafa

Title: Chief Financial Officer

[SIGNATURES CONTINUE ON THE NEXT PAGE]

[Signature Page to First Amendment Agreement]

AGENT:

HERCULES CAPITAL, INC.

Signature: /s/ Seth Meyer

Print Name: Seth Meyer

Title: CFO

[Signature Page to First Amendment Agreement]

LENDER:

HERCULES CAPITAL, INC.

Signature: /s/ Seth Meyer

Print Name: Seth Meyer

Title: CFO

HERCULES FUNDING IV LLC

Signature: /s/ Seth Meyer

Print Name: Seth Meyer

Title: Authorized Signatory

HERCULES CAPITAL FUNDING TRUST 2022-1

Signature: /s/ Seth Meyer

Print Name: Seth Meyer

Title: CFO

[Signature Page to First Amendment Agreement]

EXHIBIT A
AMENDED LOAN AGREEMENT

SECOND AMENDED AND RESTATED LOAN AND SECURITY AGREEMENT

THIS SECOND AMENDED AND RESTATED LOAN AND SECURITY AGREEMENT is made and dated as of January 6, 2023 (as amended by way of that certain First Amendment Agreement on the First Amendment Effective Date) and is entered into by and among X4 PHARMACEUTICALS, INC. a Delaware corporation (formerly known as Arsanis, Inc., the "Company"), and each of its Qualified Subsidiaries, including without limitation X4 THERAPEUTICS, INC. (formerly known as X4 Pharmaceuticals, Inc., "Therapeutics"), the several banks and other financial institutions or entities from time to time parties to this Agreement (each, a "Lender," and collectively, referred to as the "Lenders") and HERCULES CAPITAL, INC., a Maryland corporation, in its capacity as administrative agent and collateral agent for itself and the Lenders (in such capacity, the "Agent"). The Company ~~and~~, Therapeutics and each other Qualified Subsidiary that has delivered a Joinder Agreement pursuant to Section 7.13 from time to time party hereto are hereinafter referred to each, as a "Borrower," and jointly as "Borrower."

RECITALS

A. Therapeutics, as ~~Borrower~~borrower, the Lenders party thereto and Agent entered into that certain Loan and Security Agreement, dated as of October 19, 2018 (as amended by that certain Amendment No. 1 to Loan and Security Agreement, dated as of December 11, 2018, the "Original Agreement");

B. Effective as of March 13, 2019, Therapeutics merged with and into the Company's wholly owned subsidiary known as Artemis AC Corp. and was the surviving corporation of the merger with Artemis AC Corp;

C. The Company wished to become a Borrower, and Borrower, Lenders and Agent entered into an Amended and Restated Loan and Security Agreement effective as of June 27, 2019 (as subsequently amended from time to time, the "Original A&R LSA") which amended and restated the Original Agreement in its entirety, without constituting a novation;

D. As of the First Amendment Effective Date, Borrower has ~~now~~ requested the Lenders to make available to Borrower ~~a loan~~up to six (6) tranches of term loans in an aggregate principal amount of up to ~~Thirty-Two~~One Hundred Fifteen Million ~~Five Hundred Thousand~~ Dollars ($~~32,500,000.00~~115,000,000.00) (the "~~"~~Term ~~Loan"~~Loans"); and

E. The Lenders are willing to make the Term ~~Loan~~Loans on the terms and conditions set forth in this Agreement.

AGREEMENT

NOW, THEREFORE, Borrower, Agent and the Lenders agree that the Original A&R LSA is hereby amended and restated in its entirety as follows:

SECTION 1. DEFINITIONS AND RULES OF CONSTRUCTION

1.1 Unless otherwise defined herein, the following capitalized terms shall have the following meanings:

"Account Control Agreement(s)" means any agreement entered into by and among the Agent, Borrower and a third party bank or other institution (including a Securities

Intermediary) in which Borrower maintains a Deposit Account or an account holding Investment Property and which perfects Agent's first priority security interest in the subject account or accounts.

"ACH Authorization" means the ACH Debit Authorization Agreement in substantially the form of Exhibit H, which account numbers shall be redacted for security purposes if and when filed publicly by the Borrower.

"Advance(s)" means a Term Loan Advance.

"Advance Date" means the funding date of any Advance.

"Advance Request" means a request for an Advance submitted by Borrower to Agent in substantially the form of Exhibit A, which account numbers shall be redacted for security purposes if and when filed publicly by the Borrower.

"Affiliate" means, with respect to any person, (a) any Person that directly or indirectly controls, is controlled by, or is under common control with the Person in question, (b) any Person directly or indirectly owning, controlling or holding with power to vote ten percent (10%) or more of the outstanding voting securities of such Person, or (c) any Person ten percent (10%) or more of whose outstanding voting securities are directly or indirectly owned, controlled or held by such Person with power to vote such securities. As used in the definition of "Affiliate," the term "control" means the possession, directly or indirectly, of the power to direct or cause the direction of the management and policies of a Person, whether through ownership of voting securities, by contract or otherwise.

"Agent" has the meaning given to it in the preamble to this Agreement.

"Agreement" means this Second Amended and Restated Loan and Security Agreement, as amended, amended and restated, supplemented or otherwise modified from time to time.

"All Source Cash Proceeds" means unrestricted (including, not subject to any redemption, clawback, escrow or similar encumbrance or restriction) net Cash proceeds raised from one or more bona fide equity financings (which, for the avoidance of doubt, shall include cash warrant exercises), Subordinated Indebtedness, convertible Indebtedness, at-the-market (ATM) financings, upfront proceeds from corporate transactions, strategic partnerships and/or new business development transactions permitted under this Agreement, and/or Borrower's monetization of a Pediatric Review Voucher (PRV), subject to verification by Agent (including supporting documentation reasonably requested by Agent), but excluding any Cash proceeds from the Loan.

"Amortization Date" means ~~October~~April 1, ~~2024~~2025; provided however, if the Interest Only Extension Conditions are satisfied, then ~~January~~October 1, 2026~~.~~; and provided, further, that so long as (a) the Amortization Date is extended to October 1, 2026, and (b) no Event of Default exists as of October 1, 2026, then the earlier of (a) the Term Loan Maturity Date, and (b) the first day of the fiscal quarter immediately following the occurrence of an Event of Default.

"Anti-Corruption Laws" ~~shall mean~~means all laws, rules, and regulations of any jurisdiction applicable to Borrower or any of its Affiliates from time to time concerning or relating to bribery or corruption, including without limitation the United States Foreign Corrupt Practices Act of 1977, as amended, the UK Bribery Act 2010 and other similar legislation in any other jurisdictions.

"Anti-Terrorism Laws" means any laws, rules, regulations or orders relating to terrorism or money laundering, including without limitation Executive Order No. 13224 (effective September 24, 2001), the USA PATRIOT Act, the laws comprising or implementing the Bank Secrecy Act, and the laws administered by OFAC.

"Approval Milestone" means that the FDA has approved the sale and marketing of mavorixafor for the treatment to patients with Warts, Hypogammaglobulinemia, Infections and Myelokathexis (WHIM syndrome) with a label claim that is generally consistent with that sought in Borrower's New Drug Application filing, subject to Agent's reasonable verification.

"Approval Milestone Date" means the date on which Borrower achieves the Approval Milestone.

"Assignee" has the meaning given to it in Section 11.13.

"Blocked Person" means any Person: (a) listed in the annex to, or is otherwise subject to the provisions of, Executive Order No. 13224, (b) a Person owned or controlled by, or acting for or on behalf of, any Person that is listed in the annex to, or is otherwise subject to the provisions of, Executive Order No. 13224, (c) a Person with which any Lender is prohibited from dealing or otherwise engaging in any transaction by any Anti-Terrorism Law, (d) a Person that commits, threatens or conspires to commit or supports "terrorism" as defined in Executive Order No. 13224, or (e) a Person that is named a "specially designated national" or "blocked person" on the most current list published by OFAC or other similar list.

"Borrower Products" means all products, software, service offerings, technical data or technology currently being designed, manufactured or sold by Borrower or which Borrower intends to sell, license, or distribute in

the future including any products or service offerings under development, collectively, together with all products, software, service offerings, technical data or technology that have been sold, licensed or distributed by Borrower since its incorporation.

"Business Day" means any day other than Saturday, Sunday and any other day on which banking institutions in the State of California or the Commonwealth of Massachusetts are closed for business.

"Cash" means all cash, cash equivalents and liquid funds.

"Change in Control" means any reorganization, recapitalization, consolidation or merger (or similar transaction or series of related transactions) of the Company, or sale or exchange of outstanding shares (or similar transaction or series of related transactions) of Borrower, in each case in which the holders of the Company's outstanding shares immediately before consummation of such transaction or series of related transactions do not, immediately after consummation of such transaction or series of related transactions, retain shares representing more than fifty percent (50%) of the voting power of the surviving entity of such transaction or series of related transactions (or the parent of such surviving entity if such surviving entity is wholly owned by such parent), in each case without regard to whether the Company is the surviving entity, or in which the Company ceases to retain shares representing one hundred percent (100%) of the equity interests of X4 Therapeutics, Inc.

"Claims" has the meaning given to it in Section 11.10.

"Closing Date" means the date of this Agreement.

"Closing Date Facility Charge" means $162,500 (representing one-half of one percent (0.50%) of the maximum amount of the Tranche 1 Advance), due on the Closing Date.

"Code" means the Internal Revenue Code of 1986, as amended.

"Collateral" means the property described in Section 3.

"Common Stock" means the Common Stock, $0.001 par value per share, of the Company.

"~~Confidential Information~~Company" has the meaning given to it in ~~Section 11.12~~the preamble to this Agreement.

"~~Conversion Balance~~Confidential Information" has the meaning given to it in Section ~~2.2(a)~~11.12.

"Contingent Obligation" means, as applied to any Person, any direct or indirect liability, contingent or otherwise, of that Person with respect to (i) any Indebtedness, lease, dividend, letter of credit or other obligation of another, including any such obligation directly or indirectly guaranteed, endorsed, co-made or discounted or sold with recourse by that Person, or in respect of which that Person is otherwise directly or indirectly liable; (ii) any obligations with respect to undrawn letters of credit, corporate credit cards or merchant services issued for the account of that Person; and (iii) all obligations arising under any interest rate, currency or commodity swap agreement, interest rate cap agreement, interest rate collar agreement, or other agreement or arrangement designated to protect a Person against fluctuation in interest rates, currency exchange rates or commodity prices; provided, however, that the term "Contingent Obligation" shall not include endorsements for collection or deposit in the ordinary course of business. or guaranties of leases that do not constitute Indebtedness. The amount of any Contingent Obligation shall be deemed to

be an amount equal to the stated or determined amount of the primary obligation in respect of which such Contingent Obligation is made or, if not stated or determinable, the maximum reasonably anticipated liability in respect thereof as determined by such Person in good faith; provided, however, that such amount shall not in any event exceed the maximum amount of the obligations under the guarantee or other support arrangement.

"Conversion Balance" has the meaning given to it in Section 2.2(a).

"Copyright License" means any written agreement granting any right to use any Copyright or Copyright registration, now owned or hereafter acquired by Borrower or in which Borrower now holds or hereafter acquires any interest.

"Copyrights" means all copyrights, whether registered or unregistered, held pursuant to the laws of the United States of America, any State thereof, or of any other country.

"Default" means any event, circumstance or condition that has occurred or exists, that would, with the passage of time or the requirement that notice be given or both, become an Event of Default.

"Deposit Accounts" means any "deposit accounts," as such term is defined in the UCC, and includes any checking account, savings account, or certificate of deposit.

"Domestic Subsidiary" means any Subsidiary that is not a Foreign Subsidiary.

"Eligible Foreign Subsidiary" means any Foreign Subsidiary other than an Excluded Subsidiary, whose execution of a Joinder Agreement could not result in a material adverse tax consequence to Borrower.

"End of Term Charge" has the meaning given to it in Section 2.6(c).

"End of Term Charge I" has the meaning given to it in Section 2.6(a).

"End of Term Charge II" has the meaning given to it in Section 2.6(b).

"End of Term Charge III" has the meaning given to it in Section 2.6(c).

"Equity Interests" means, with respect to any Person, the capital stock, partnership or limited liability company interest, or other equity securities or equity ownership interests of such Person.

"ERISA" means the Employee Retirement Income Security Act of 1974, as amended, and the regulations promulgated thereunder.

"Event of Default" has the meaning given to it in Section 9.

"Excluded Account" means any "zero balance" deposit account or securities account used exclusively for payroll, employee benefits or employee taxes, the funds of which shall not exceed the amount required to pay the next payroll or other relevant cycle, and identified to the Agent in writing by the Borrower as such.

"Excluded Subsidiary" means X4 Pharmaceuticals (Austria) GmbH.

~~"Facility Charge" means $162,500 (representing one-half percent (0.50%) of the maximum amount of the Term Loan), due on the Closing Date.~~

"FDA" means the U.S. Food and Drug Administration or any successor thereto.

"Financial Statements" has the meaning given to it in Section 7.1.

"First Amendment Agreement" means that certain First Amendment to Second Amended and Restated Loan and Security Agreement dated August 2, 2023 by and among the Borrowers, the Lenders and the Agent.

"First Amendment Effective Date" shall have the meaning given to such term in the First Amendment Agreement.

"Foreign Subsidiary" means any Subsidiary other than a Subsidiary organized under the laws of any state within the United States of America.

"GAAP" means generally accepted accounting principles in the United States of America, as in effect from time to time.

"Indebtedness" means indebtedness of any kind, including (a) all indebtedness for borrowed money or the deferred purchase price of property or services (excluding trade credit entered into in the ordinary course of business due within ninety (90) days), including reimbursement and other obligations with respect to surety bonds and letters of credit, (b) all obligations evidenced by notes, bonds, debentures or similar instruments, (c) all capital lease obligations, (d) equity securities of any Person subject to repurchase or redemption other than at the sole option of such Person and (e) all Contingent Obligations within the meaning of GAAP as in effect on the Closing Date; provided that Indebtedness shall not include endorsements of checks or drafts arising in the ordinary course of business.

~~"Insolvency Proceeding" is any proceeding by or against any Person under the United States Bankruptcy Code, or any other bankruptcy or insolvency law, including assignments for the benefit of creditors, compositions, extensions generally with its creditors, or proceedings seeking reorganization, arrangement, or other similar relief.~~

"Intellectual Property" means all of Borrower's Copyrights; Trademarks; Patents; Licenses; trade secrets and inventions; mask works; Borrower's applications therefor and reissues, extensions, or renewals thereof; and Borrower's goodwill associated with any of the foregoing, together with Borrower's rights to sue for past, present and future infringement of Intellectual Property and the goodwill associated therewith.

"Interest Only Extension Conditions" shall mean satisfaction of each of the following events: (a) no ~~default~~Default or Event of Default shall have occurred~~;~~, and (b~~) Borrower shall have obtained a minimum of $110,000,000 in All Source Cash Proceeds during the period beginning January 1, 2023 through December 31, 2024; and (c~~) the Approval Milestone Date shall have occurred on or before ~~December 31, 2024~~September 30, 2026.

"Inventory" means "inventory" as defined in Article 9 of the UCC.

"Investment" means (a) any beneficial ownership (including stock, partnership, limited liability company interests, or other securities) of or in any Person, (b) any loan, advance or capital contribution to any Person, (c) or the acquisition of any asset of another Person not in the ordinary course of business.

~~"IRS" means the United States Internal Revenue Service.~~

"Joinder Agreements" means for each Qualified Subsidiary, a completed and executed Joinder Agreement in substantially the form attached hereto as Exhibit G.

"Lender" and "Lenders" have the meaning given to it in the preamble to this Agreement.

"License" means any Copyright License, Patent License, Trademark License or other license of rights or interests.

"Lien" means any mortgage, deed of trust, pledge, hypothecation, assignment for security, security interest, encumbrance, levy, lien or charge of any kind, whether voluntarily incurred or arising by operation of law or otherwise, against any property, any conditional sale or other title retention agreement, and any lease in the nature of a security interest.

"Loan" means the Advances made under this Agreement.

"Loan Documents" means this Agreement, the Notes (if any), the ACH Authorization, the Account Control Agreements, the Joinder Agreements, all UCC Financing Statements, the Pledge Agreements, the Warrants issued in connection with the Original Agreement and any other documents executed in connection with the Secured Obligations or the transactions contemplated hereby, as the same may from time to time be amended, modified, supplemented or restated.

"Market Capitalization" means, as of any date of determination, the product of (a) the sum of (i) the number of outstanding shares of Common Stock publicly disclosed in the most recent filing of Company with the Securities and Exchange Commission as outstanding as of such date of determination ~~and~~, *plus* (ii) the number of shares of Common Stock that could be claimed through the exercise of outstanding warrants publicly disclosed in the most recent filing of Company with the Securities and Exchange Commission as of such date of determination, *plus* (iii) the number of shares of Common Stock that could be claimed through the exercise of outstanding options publicly disclosed in the most recent filing of Company with the Securities and Exchange Commission as of such date of determination, *plus* (iv) the number of shares of Common Stock that could be claimed through the settlement of outstanding restricted stock units publicly disclosed in the most recent filing of Company with the Securities and Exchange Commission as of such date of determination, *multiplied by* (b) the most recent closing price of Company's Common Stock (as quoted on Bloomberg L.P.'s page or any successor page thereto of Bloomberg L.P. or if such page is not available, any other commercially available source).

"Material Adverse Effect" means a material adverse effect upon: (i) the business, operations, properties, assets or financial condition of Borrower and its Subsidiaries taken as a whole; or (ii) the ability of Borrower to perform or pay the Secured Obligations in accordance with the terms of the Loan Documents, or the ability of Agent or the Lenders to enforce any of its rights or remedies with respect to the Secured Obligations; or (iii) the Collateral or Agent's Liens on the Collateral or the priority of such Liens.

"Maximum Rate" shall have the meaning assigned to such term in Section 2.3.

"Net Product Revenue~~'~~" means revenue solely generated from the sale of mavorixafor (as determined in accordance with GAAP), which shall include royalty, profit sharing, co-development and co-promotion-commercialization revenues but shall exclude milestone payments and any other one-time revenue.

"Net Product Revenue Forecast" means for Borrower's 2025 fiscal year, the forecast of Borrower's Net Product Revenue based on the financial projections provided to Agent prior to the Closing Date, with the forecast for

future years based on projections approved by Borrower's board of directors in accordance with Section 7.1(h) hereof and reasonably acceptable to Agent.

"New Drug Application" means an application submitted to the FDA pursuant to 21 U.S.C. § 355 seeking authorization to market a new drug in the United States.

"Non-Disclosure Agreement" means that certain Confidential Disclosure Agreement by and between X4 Pharmaceuticals, Inc. and Hercules Capital, Inc. dated as of August 14, 2018.

"Note(s)" means a Term Note.

"OFAC" is the U.S. Department of Treasury Office of Foreign Assets Control.

"OFAC Lists" are, collectively, the Specially Designated Nationals and Blocked Persons List maintained by OFAC pursuant to Executive Order No. 13224, 66 Fed. Reg. 49079 (Sept. 25, 2001) and/or any other list of terrorists or other restricted Persons maintained pursuant to any of the rules and regulations of OFAC or pursuant to any other applicable Executive Orders.

~~"Original Closing Date" means October 19, 2018.~~

"Original Agreement" has the meaning given to it in the Recitals.

"Original A&R LSA" has the meaning given to it in the Recitals.

"Original A&R LSA Closing Date" means June 27, 2019.

"Original A&R LSA Term Loans" has the meaning given to it in Section 2.2(a).

"Participant Register" shall have the meaning assigned to such term in Section 11.21.

"Patent License" means any written agreement granting any right with respect to any invention on which a Patent is in existence or a Patent application is pending, in which agreement Borrower now holds or hereafter acquires any interest.

"Patents" means all letters patent of, or rights corresponding thereto, in the United States of America or in any other country, all registrations and recordings thereof, and all applications for letters patent of, or rights corresponding thereto, in the United States of America or any other country.

"Permitted Acquisition" ~~shall mean~~means any acquisition (including by way of merger or exclusive in-licensing arrangements) by Borrower of all or substantially all of the assets of another Person, or of a division or line of business of another Person, or capital stock of another Person, in each case located entirely within the United States of America, which is conducted in accordance with the following requirements:

(a) if such acquisition is of a business or Person, such business or Person is engaged in a line of business similar or related to that of the Borrower or its Subsidiaries;

(b) if such acquisition is structured as a stock acquisition, then the Person so acquired shall either (i) become a wholly-owned Subsidiary of Borrower or of a Subsidiary and the Borrower shall comply, or cause such

Subsidiary to comply, with 7.13 hereof or (ii) such Person shall be merged with and into Borrower (with the Borrower being the surviving entity);

(c) if such acquisition is structured as the acquisition of assets, such assets shall be acquired by Borrower, and shall be free and clear of Liens other than Permitted Liens;

(d) the Borrower shall have delivered to Lender not less than ten (10) nor more than forty five (45) days prior to the date of such acquisition, notice of such acquisition together with pro forma projected financial information, copies of all material documents relating to such acquisition, and historical financial statements for such acquired entity, division or line of business, in each case in form and substance reasonably satisfactory to Lender and demonstrating compliance with the covenants set forth in Section 7 hereof on a pro forma basis as if the acquisition occurred on the first day of the most recent measurement period;

(e) both immediately before and after such acquisition no Default or Event of Default shall have occurred and be continuing; and

(f) the sum of the purchase price of such proposed new acquisition, computed on the basis of total acquisition consideration paid or incurred, or to be paid or incurred (but excluding for such purpose any performance-based milestones, earn-outs, royalties or similar payments), by Borrower with respect thereto, including the amount of Permitted Indebtedness assumed or to which such assets, businesses or business or ownership interest or shares, or any Person so acquired, is subject, shall not be greater than (i) $5,000,000 for any single acquisition or group of related acquisitions or (ii) $5,000,000 for all such acquisitions during the term of the Loan.

"Permitted Indebtedness" means: (i) Indebtedness of Borrower in favor of the Lenders or Agent arising under this Agreement or any other Loan Document; (ii) Indebtedness existing on the Closing Date which is disclosed in Schedule 1A; (iii) Indebtedness of up to $1,000,000 outstanding at any time secured by a Lien described in clause (vii) of the defined term "Permitted Liens," provided such Indebtedness does not exceed the cost of the Equipment financed with such Indebtedness; (iv) Indebtedness to trade creditors incurred in the ordinary course of business, and Indebtedness incurred in the ordinary course of business with corporate credit cards; (v) Indebtedness that also constitutes a Permitted Investment; (vi) Subordinated Indebtedness; (vii) reimbursement obligations in connection with letters of credit that are secured by Cash and issued on behalf of the Borrower or a Subsidiary thereof in an amount not to exceed $400,000 at any time outstanding, (viii) other unsecured Indebtedness in a principal amount not to exceed $300,000 at any time outstanding, (ix) intercompany Indebtedness as long as either (A) each of the Subsidiary obligor and the Subsidiary obligee under such Indebtedness is a Qualified Subsidiary that has executed a Joinder Agreement and (x) extensions, amendments, restatements, refinancings and renewals of any items of Permitted Indebtedness, provided that the principal amount is not increased or the terms modified to impose materially more burdensome terms upon Borrower or its Subsidiary, as the case may be.

"Permitted Investment" means: (i) Investments existing on the Closing Date which are disclosed in Schedule 1B; (ii) (a) marketable direct obligations issued or unconditionally guaranteed by the United States of America or any agency or any State thereof maturing within one year from the date of acquisition thereof currently having a rating of at least A-2 or P-2 from either Standard & Poor's Corporation or Moody's Investors Services, (b) commercial paper maturing no more than one year from the date of creation thereof and currently having a rating of at least A-2 or P-2 from either Standard & Poor's Corporation or Moody's Investors Service, (c) certificates of deposit issued by any bank with assets of at least $500,000,000 maturing no more than one year from the date of investment therein, (d) money market accounts, and (e) Investments made pursuant to the investment policy guidelines of the Borrower in

effect as of the Closing Date or amended guidelines as approved by Borrower's Board of Directors; (iii) repurchases of stock from current or former employees, directors, or consultants of Borrower under the terms of applicable repurchase agreements at the original issuance price of such securities in an aggregate amount not to exceed $375,000 in any fiscal year, provided that no Event of Default has occurred and is continuing or would immediately result after giving effect to the repurchases; (iv) Investments consisting of the endorsement of negotiable instruments for deposit or collection or similar transactions in the ordinary course of business; (v) Investments accepted in connection with Permitted Transfers; (vi) Investments (including debt obligations) received in connection with the bankruptcy or reorganization of customers or suppliers and in settlement of delinquent obligations of, and other disputes with, customers or suppliers arising in the ordinary course of Borrower's business; (vii) Investments consisting of notes receivable of, or prepaid royalties and other credit extensions, to customers and suppliers who are not Affiliates, in the ordinary course of business, provided that this subparagraph (vii) shall not apply to Investments of Borrower in any Subsidiary; (viii) Investments consisting of loans not involving the net transfer on a substantially contemporaneous basis of cash proceeds to employees, officers or directors relating to the purchase of capital stock of Borrower pursuant to employee stock purchase plans or other similar agreements approved by Borrower's Board of Directors; (ix) Investments consisting of travel advances, employee relocation loans, and other employee loans and advances in the ordinary course of business in an aggregate amount not to exceed $375,000 in any fiscal year or $750,000 during the term hereof; (x) Investments in newly-formed Domestic Subsidiaries, provided that each such Domestic Subsidiary, other than Security Corporation Subsidiary, enters into a Joinder Agreement promptly after its formation by Borrower and execute such other documents as shall be

reasonably requested by Agent; (xi) Investments in Foreign Subsidiaries approved in advance in writing by Agent; (xii) so long as no Event of Default has occurred and is continuing, Investments in the Excluded Subsidiary from time to time on an ongoing basis, sufficient to permit the Excluded Subsidiary to operate and conduct its business in the ordinary course; (xiii) joint ventures or strategic alliances in the ordinary course of Borrower's business consisting of the licensing of technology, the development of technology or the providing of technical support, provided that any cash Investments by Borrower do not exceed $750,000 in the aggregate in any fiscal year; (xiv) Investments in Permitted Acquisitions; (xv) Investments in Security Corporation Subsidiary; and (xvi) additional Investments that do not exceed $500,000 in the aggregate.

"Permitted Liens" means any and all of the following: (i) Liens in favor of Agent or the Lenders; (ii) Liens existing on the Closing Date which are disclosed in Schedule 1C; (iii) Liens for taxes, fees, assessments or other governmental charges or levies, either not delinquent or being contested in good faith by appropriate proceedings; provided, that Borrower maintains adequate reserves therefor in accordance with GAAP (to the extent required hereby); (iv) Liens securing claims or demands of materialmen, artisans, mechanics, carriers, warehousemen, landlords and other like Persons arising in the ordinary course of Borrower's business and imposed without action of such parties; provided, that the payment thereof is not yet required; (v) Liens arising from judgments, decrees or attachments in circumstances which do not constitute an Event of Default hereunder; (vi) deposits to secure the performance of obligations not to exceed $200,000 in the aggregate, and the following deposits, to the extent made in the ordinary course of business: deposits under worker's compensation, unemployment insurance, social security and other similar laws, or to secure the performance of bids, tenders or contracts (other than for the repayment of borrowed money) or to secure indemnity, performance or other similar bonds for the performance of bids, tenders or contracts (other than for the repayment of borrowed money) or to secure statutory obligations (other than Liens arising under ERISA or environmental Liens) or surety or appeal bonds, or to secure indemnity, performance or other similar bonds; (vii) Liens on Equipment or software or other intellectual property constituting purchase money Liens and Liens in connection with

capital leases securing Indebtedness permitted in clause (iii) of "Permitted Indebtedness"; (viii) Liens incurred in connection with Subordinated Indebtedness; (ix) leasehold interests in leases or subleases and licenses or sublicenses granted in the ordinary course of business and not interfering in any material respect with the business of the lessor licensor, as applicable; (x) Liens in favor of customs and revenue authorities arising as a matter of law to secure payment of custom duties that are promptly paid on or before the date they become due; (xi) Liens on insurance proceeds securing the payment of financed insurance premiums that are promptly paid on or before the date they become due (provided that such Liens extend only to such insurance proceeds and not to any other property or assets); (xii) statutory and common law rights of set-off and other similar rights as to deposits of cash and securities in favor of banks, other depository institutions and brokerage firms; (xiii) easements, zoning restrictions, rights-of-way and similar encumbrances on real property imposed by law or arising in the ordinary course of business so long as they do not materially impair the value or marketability of the related property; (xiv) (A) Liens on Cash securing obligations permitted under clause (vii) of the definition of Permitted Indebtedness and (B) security deposits in connection with real property leases, the combination of (A) and (B) in an aggregate amount not to exceed $750,000 at any time; (xv) Liens incurred in connection with the extension, renewal or refinancing of the Indebtedness secured by Liens of the type described in clauses (i) through (xi) above; provided, that any extension, renewal or replacement Lien shall be limited to the property encumbered by the existing Lien and the principal amount of the Indebtedness being extended, renewed, refinanced, modified, amended, restated or amended and restated (as may have been reduced by any payment thereon) does not increase; (xvi) Liens in connection with "precautionary filings" in connection with operating leases of the Equipment that is the subject of such leases; provided that such Liens and collateral descriptions in such precautionary filings are limited to such specific operating leases and not all assets or substantially all assets of the Borrower or any Subsidiary; and (xvii) other Liens securing obligations not to exceed the principal amount of $500,000 outstanding at any time.

"Permitted Transfers" means (i) sales of Inventory in the ordinary course of business, (ii) non-exclusive outbound licenses and similar arrangements for the use of Intellectual Property in the ordinary course of business, (iii) exclusive licenses for the use of the Intellectual Property of Borrower or Borrower Products in the field of Immuno-Oncology entered into the ordinary course of business provided that each such license constitutes an arms-length transaction, that could not result in a legal transfer of title of the licensed property, and so long as after giving effect to each such non-exclusive or exclusive license, Borrower and its Subsidiaries retain sufficient rights to use or benefit from the subject Intellectual Property as to enable them to conduct their business in the ordinary course, (iv) dispositions of worn-out, obsolete or surplus Equipment at fair market value in the ordinary course of business, (v) transfers consisting of Permitted Investments or Permitted Liens, (vi) transfers of Cash in the ordinary course of business to the extent not otherwise inconsistent with the terms and conditions of this Agreement, and (vii) other Transfers of assets having a fair market value of not more than $500,000 in the aggregate in any fiscal year.

"Person" means any individual, sole proprietorship, partnership, joint venture, trust, unincorporated organization, association, corporation, limited liability company, institution, other entity or government.

"Pledge Agreements" means the Amended and Restated Pledge Agreement dated as of the Original A&R LSA Closing Date between Therapeutics and Agent, as the same may from time to time be amended, restated, modified, assumed or otherwise supplemented, and the Pledge Agreement dated as of the Original A&R LSA Closing Date between the Company and Agent, as the same may from time to time be amended, restated, modified, assumed or otherwise supplemented.

"Prepayment Charge" shall have the meaning assigned to such term in Section 2.5.

"Prime Rate" means the "prime rate" as reported in *The Wall Street Journal* or any successor publication thereto.

"Qualified Cash" means the amount of Borrower's Cash and Cash equivalents held in accounts subject to an Account Control Agreement in favor of Agent.

"Qualified Subsidiary" means any direct or indirect Domestic Subsidiary (other than Security Corporation Subsidiary) or Eligible Foreign Subsidiary.

"Receivables" means (i) all of Borrower's Accounts, Instruments, Documents, Chattel Paper, Supporting Obligations, letters of credit, proceeds of any letter of credit, and Letter of Credit Rights, and (ii) all customer lists, software, and business records related thereto.

"Register" has the meaning specified in Section 11.7.

"Required Lenders" means at any time, the holders of more than 50% of the aggregate unpaid principal amount of the Term Loans then outstanding.

"Sanctioned Country" shall mean, at any time, a country or territory which is the subject or target of any Sanctions.

"Sanctioned Person" shall mean, at any time, (a) any Person listed in any Sanctions-related list of designated Persons maintained by the Office of Foreign Assets Control of the U.S. Department of the Treasury or the U.S. Department of State, or by the United Nations Security Council, the European Union or any EU member state, (b) any Person operating, organized or resident in a Sanctioned Country or (c) any Person controlled by any such Person.

"Sanctions" shall mean economic or financial sanctions or trade embargoes imposed, administered or enforced from time to time by (a) the U.S. government, including those administered by the Office of Foreign Assets Control of the U.S. Department of the Treasury or the U.S. Department of State, or (b) the United Nations Security Council, the European Union or Her Majesty's Treasury of the United Kingdom.

"SEC" means the Securities and Exchange Commission, or any successor thereto.

"Secured Obligations" means Borrower's obligations under this Agreement and any Loan Document (other than the Warrants), including without limitation the Pledge Agreements, and including any obligation to pay any amount now owing or later arising.

"Security Corporation Investment Conditions" means that Borrower maintains Qualified Cash in an aggregate amount greater than or equal to the lesser of (i) 110% of the aggregate principal amount of Term Loan Advances outstanding under this Agreement and (ii) 100% of the Company and its consolidated Subsidiaries' unrestricted Cash reserves, unless compliance with the foregoing conditions is waived in writing from time to time by Agent with respect to specified periods, in Agent's sole discretion.

"Security Corporation Subsidiary" means X4 Pharmaceuticals Securities Corporation, a wholly-owned Subsidiary incorporated in the Commonwealth of Massachusetts or the State of Delaware for the purpose of holding Investments as a Massachusetts security corporation under 830 CMR 63.38B.1 of the Massachusetts tax code and applicable regulations (as the same may be amended, modified or replaced from time to time).

"Subordinated Indebtedness" means Indebtedness subordinated to the Secured Obligations in amounts and on terms and conditions satisfactory to Agent in its sole discretion and subject to a written subordination agreement in form and substance satisfactory to Agent in its sole discretion.

"Subsequent Financing" means the closing by the Company after the Closing Date of the issuance and sale of the Company's equity securities for cash solely for financing purposes in an offering broadly marketed to multiple investors, which shall not include the issuance and sale by the Company of its equity securities (i) pursuant to benefit plans or arrangements, including under the Company's equity incentive plans or otherwise as equity compensation, (ii) as dividends or distributions or upon stock splits, recapitalizations or similar transactions, (iii) pursuant to a merger, consolidation, acquisition, strategic alliance or similar business combination or acquisition, (iv) to banks, equipment or real property lessors or other financial institutions pursuant to a non-convertible debt financing, equipment lease, bank credit arrangement or commercial leasing transaction entered into for primarily non-equity financing purposes, (v) in connection with strategic transactions, including (A) joint ventures, manufacturing, marketing, OEM, sponsored research, collaboration or distribution arrangements or (B) technology transfer or development arrangements, (vi) securities issued or issuable to suppliers or third party service providers in connection with the provision of goods or services, (vii) in an at-the-market (ATM) offering, (viii) securities issued to acquire any security convertible into the securities excluded from the definition of Subsequent Financing pursuant to clause (i) through (vii) above and (ix) securities issued in connection with options, warrants, convertible securities or other arrangement in existence on the Closing Date or issued in

transactions excluded from the definition of Subsequent Financing pursuant to clause (i) through (viii) above; provided, however, that, if the Company or its agents attempts to "wall-cross" the Lender or its assignee or nominee in conjunction with any Subsequent Financing and the Lender or its assignee or nominee declines to be "wall-crossed," then the issuance and sale of such equity securities shall not be considered a Subsequent Financing hereunder.

"Subsidiary" means an entity, whether corporate, partnership, limited liability company, joint venture or otherwise, in which Borrower owns or controls 50% or more of the outstanding voting securities, including each entity listed on Schedule 1 hereto.

"T6M Net Product Revenue" means Borrower's Net Product Revenue measured on a trailing six month basis.

"Term Commitment" means as to any Lender, the obligation of such Lender, if any, to make a Term Loan Advance to the Borrower in a principal amount not to exceed the amount set forth under the heading "~~Term~~Tranche 1 Commitment", "Tranche 2 Commitment", "Tranche 3 Commitment", "Tranche 5 Commitment" or "Tranche 6 Commitment", as the case may be, opposite such Lender's name on Schedule 1.1.

~~"Term Loan" means a loan in an aggregate principal amount of up to Thirty-Two Million Five Hundred Thousand Dollars ($32,500,000).~~

"Term Loan Advance" ~~has the meaning assigned to such term in~~means each Tranche 1 Advance, Tranche 2 Advance, Tranche 3 Advance, Tranche 4 Advance, Tranche 5 Advance, Tranche 6 Advance, and any other funds advanced under Section 2.2(a)~~, and any other Term Loan funds advanced under this Agreement~~.

"Term Loan Interest Rate" means for any day a per annum rate of interest equal to the greater of either (a) (i) 3.15% *plus*~~the prime rate as reported in The Wall Street Journal and (ii)~~(ii) the Prime Rate, and (b) 10.15%.

"Term Loan Maturity Date" means ~~April~~October 1, 2026; provided however, if the Interest Only Extension Conditions are achieved, then July 1, 2027.

"Term Loans" has the meaning given to it in the recitals to this Agreement.

"Term Note" means a Promissory Note in substantially the form of Exhibit B.

"Trademark License" means any written agreement granting any right to use any Trademark or Trademark registration, now owned or hereafter acquired by Borrower or in which Borrower now holds or hereafter acquires any interest.

"Trademarks" means all trademarks (registered, common law or otherwise) and any applications in connection therewith, including registrations, recordings and applications in the United States Patent and Trademark Office or in any similar office or agency of the United States of America, any State thereof or any other country or any political subdivision thereof.

"Tranche" means the Tranche 1 Advance, the Tranche 2 Advance, the Tranche 3 Advance, the Tranche 4 Advance, the Tranche 5 Advance, and/or any Tranche 6 Advance, as applicable.

"Tranche 1 Advance" has the meaning given to it in Section 2.2(a)(i).

"Tranche 1 Term Commitment" means as to any Lender, the obligation of such Lender, if any, to make a Term Loan Advance to Borrower in a principal amount not to exceed the amount set forth under the heading Tranche 1 Commitment opposite such Lender's name on Schedule 1.1.

"Tranche 2 Advance" has the meaning given to it in Section 2.2(a)(ii).

"Tranche 3 Advance" has the meaning given to it in Section 2.2(a)(iii).

"Tranche 4 Advance" has the meaning given to it in Section 2.2(a)(iv).

"Tranche 5 Advance" has the meaning given to it in Section 2.2(a)(v).

"Tranche 5 Milestone Date" means the first date on which Borrower has provided evidence to Agent, satisfactory to Agent in its reasonable discretion, that: (i) no Default or Event of Default is continuing, (ii) the Tranche 4 Advance has been drawn in full, and (iii) one or more patients have received an evaluated dose of mavorixafor in a registration-directed trial designed to evaluate the safety and efficacy of mavorixafor as a treatment for patients diagnosed with chronic neutropenia.

"Tranche 6 Advance" has the meaning given to it in Section 2.2(a)(vi).

"Tranche 6 Facility Charge" means one-half of one percent (0.50%) of any Tranche 6 Advance, which is payable to Lenders in accordance with Section 4.2(e).

"UCC" means the Uniform Commercial Code as the same is, from time to time, in effect in the State of California; provided, that in the event that, by reason of mandatory provisions of law, any or all of the attachment, perfection or priority of, or remedies with respect to, Agent's Lien on any Collateral is governed by the Uniform Commercial Code as the same is, from time to time, in effect in a jurisdiction other than the State of California, then the

term “UCC” shall mean the Uniform Commercial Code as in effect, from time to time, in such other jurisdiction solely for purposes of the provisions thereof relating to such attachment, perfection, priority or remedies and for purposes of definitions related to such provisions.

“Warrants” means that certain Amended and Restated Warrant Agreement dated as of March 29, 2019 and that certain Warrant Agreement dated as of March 18, 2019, entered into by the Company in connection with the Original Agreement, as such agreements may be amended, restated or modified from time to time.

1.2 Unless otherwise specified, all references in this Agreement or any Annex or Schedule hereto to a “Section,” “subsection,” “Exhibit,” “Annex,” or “Schedule” shall refer to the corresponding Section, subsection, Exhibit, Annex, or Schedule in or to this Agreement. Unless otherwise specifically provided herein, any accounting term used in this Agreement or the other Loan Documents shall have the meaning customarily given such term in accordance with GAAP, and all financial computations hereunder shall be computed in accordance with GAAP, consistently applied. Unless otherwise defined herein or in the other Loan Documents, terms that are used herein or in the other Loan Documents and defined in the UCC shall have the meanings given to them in the UCC. For all purposes under the Loan Documents, in connection with any division or plan of division under Delaware law (or any comparable event under a different jurisdiction’s laws): (a) if any asset, right, obligation or liability of any Person becomes the asset, right, obligation or liability of a different Person, then it shall be deemed to have been transferred from the original Person to the subsequent Person and (b) if any new Person comes into existence, such new Person shall be deemed to have been organized on the first date of its existence by the holders of its Equity Interests at such time.

SECTION 2. THE LOAN

2.1 [Intentionally omitted.]

2.2 Term ~~Loan~~Loans.

(a)Term Loan Advances.

(~~a~~i) ~~Term Loan Advances~~Tranche 1. Pursuant to the Original A&R LSA, the Lenders party thereto extended ~~“~~“Term Loans~~”~~” (under and as defined in the A&R LSA) (the ~~“~~“Original A&R LSA Term Loans~~”~~”) to Borrower in the original aggregate principal amount of Thirty-Two Million Five Hundred Thousand Dollars ($32,500,000). Borrower acknowledges and agrees that, as of the Closing Date, Thirty-Two Million Five Hundred Thousand Dollars ($32,500,000) (the “Conversion Balance”) of the principal amount of the Original A&R LSA Term Loans remains outstanding and such entire outstanding principal balance shall for all purposes hereunder be deemed to constitute and be referred to, and hereby is converted into, a Term Loan Advance in like amount hereunder, without constituting a novation, and shall be deemed an Advance on the Closing Date for purposes of this Agreement. Subject to the terms and conditions of this Agreement, the Lenders will severally (and not jointly) make in an amount not to exceed their respective Tranche 1 Term Commitments, and Borrower agrees to draw, such Term Loan Advance of Thirty-Two Million Five Hundred Thousand Dollars ($32,500,000) (the “~~Term Loan~~Tranche 1 Advance”), inclusive of the Conversion Balance on the Closing Date.

(ii)Tranche 2. Subject to the terms and conditions of this Agreement, on the First Amendment Effective Date, Lenders will severally (and not jointly) make, and Borrower agrees to draw, a Term Loan

Advance in an aggregate principal amount equal to Twenty-Two Million Five Hundred Thousand Dollars ($22,500,000) (such Term Loan Advance, the "Tranche 2 Advance").

(iii)Tranche 3. Subject to the terms and conditions of this Agreement, Borrower may request, and the Lenders shall severally (and not jointly) make, in each case, beginning on the Approval Milestone Date and continuing through the earlier of (A) September 30, 2024 and (B) the date that is forty-five (45) days after the Approval Milestone Date, a Term Loan Advance in an aggregate principal amount of up to Twenty Million Dollars ($20,000,000); provided that such Term Loan Advance shall not be less than Ten Million Dollars ($10,000,000) (such Term Loan Advance, the "Tranche 3 Advance").

(iv)Tranche 4. Subject to the terms and conditions of this Agreement, Borrower may request, and the Lenders shall severally (and not jointly) make, in each case, beginning on the Advance Date for the Tranche 3 Advance and continuing through December 15, 2024, a Term Loan Advance in an aggregate principal amount equal to Twenty Million Dollars ($20,000,000) *less* the aggregate principal amount of any Tranche 3 Advance (such Term Loan Advance, the "Tranche 4 Advance").

(v)Tranche 5. Subject to the terms and conditions of this Agreement, Borrower may request, and the Lenders shall severally (and not jointly) make, in each case, beginning on the Tranche 5 Milestone Date and continuing through the earlier of (A) December 15, 2024 and (B) the date that is forty-five (45) days after the Tranche 5 Milestone Date, a Term Loan Advance in an aggregate principal

amount equal to Seven Million Five Hundred Thousand Dollars ($7,500,000) (such Term Loan Advance, the "Tranche 5 Advance").

(vi)Tranche 6. Subject to the terms and conditions of this Agreement, Borrower may request, and the Lenders shall severally (and not jointly) make, in each case, conditioned on approval by Lenders' investment committee in its sole and unfettered discretion, one or more additional Term Loan Advances in minimum increments of Ten Million Dollars ($10,000,000) (or if less, the remaining amount of Term Loan Advances available to be drawn pursuant to this Section 2.2(a)(vi)) in an aggregate principal amount up to Thirty Two Million Five Hundred Thousand Dollars ($32,500,000) (such Term Loan Advances, the "Tranche 6 Advances").

(b) Advance Request. To obtain a Term Loan Advance, Borrower shall complete, sign and deliver an Advance Request (at least one (1) Business Day before the Closing Date or the First Amendment Effective Date, and at least five (5) Business Days before each Advance Date other than the Closing Date or the First Amendment Effective Date) to Agent. The Lenders shall fund the Term Loan Advance in the manner requested by the Advance Request provided that each of the conditions precedent to such Term Loan Advance is satisfied as of the requested Advance Date. The proceeds of any Term Loan Advance shall be deposited into an account that is subject to an Account Control Agreement.

(c) Term Loan Interest Rate. The principal balance of each Term Loan Advance shall bear interest thereon from such Advance Date at the Term Loan Interest Rate based on a year consisting of 360 days, with interest computed daily based on the actual number of days elapsed. The Term Loan Interest Rate will float and change on the day the ~~prime rate~~Prime Rate changes from time to time. Notwithstanding anything to the contrary in this Agreement, the outstanding principal balance of the Original A&R LSA Term Loans shall have accrued interest at the "Term Loan Interest Rate" (as defined in the Original A&R LSA) through the date immediately

preceding the Closing Date, and any such accrued but unpaid interest shall be due and payable on the next scheduled interest payment date following the Closing Date pursuant to Section 2.2(d).

(d) Payment. Borrower will pay accrued but unpaid interest on each Term Loan Advance on the first Business Day of each month (each such date, a "Payment Date"), beginning the month after the corresponding Advance Date. Borrower shall repay the aggregate ~~Term Loan~~ principal balance of the Term Loan Advances that is outstanding on the day immediately preceding the Amortization Date, in equal monthly installments of principal and interest (mortgage style) beginning on the Amortization Date and continuing on the first Business Day of each month thereafter until the Secured Obligations (other than inchoate indemnity obligations and other obligations that are stated to survive termination of this Agreement) are repaid. The entire ~~Term Loan~~ principal balance of the Term Loan Advances and all accrued but unpaid interest hereunder, shall be due and payable on the Term Loan Maturity Date. Borrower shall make all payments under this Agreement without setoff, recoupment or deduction and regardless of any counterclaim or defense. If a payment hereunder becomes due and payable on a day that is not a Business Day, the due date thereof shall be the immediately preceding Business Day. ~~The~~Agent or the Lenders will initiate debit entries to the Borrower's account as authorized on the ACH Authorization (i) on each ~~payment date~~Payment Date of all periodic obligations payable to the Lenders under each Term Loan Advance and (ii) reasonable and documented out-of-pocket legal fees and costs incurred by Agent or the Lenders in connection with Section 11.11 of this Agreement; provided that, with respect to clause (i) above, in the event that the Lenders or Agent

informs Borrower that the Lenders will not initiate a debit entry to Borrower's account for a certain amount of the periodic obligations due on a specific ~~payment date~~Payment Date, Borrower shall pay to the Lenders such amount of periodic obligations in full in immediately available funds on such payment date; provided, further, that, with respect to clause (i) above, if the Lenders or Agent informs Borrower that the Lenders will not initiate a debit entry as described above later than the date that is three (3) Business Days prior to such payment date, Borrower shall pay to the Lenders such amount of periodic obligations in full in immediately available funds on the date that is three (3) Business Days after the date on which the Lenders or Agent notifies Borrower of such; provided, further, that, with respect to clause (ii) above, in the event that the Lenders or Agent informs Borrower that the Lenders will not initiate a debit entry to Borrower's account for certain amount of such out-of-pocket legal fees and costs incurred by Agent or the Lenders, Borrower shall pay to the Lenders such amount in full in immediately available funds within three (3) Business Days.

2.3 Maximum Interest. Notwithstanding any provision in this Agreement or any other Loan Document, it is the parties' intent not to contract for, charge or receive interest at a rate that is greater than the maximum rate permissible by law that a court of competent jurisdiction shall deem applicable hereto (which under the laws of the State of California shall be deemed to be the laws relating to permissible rates of interest on commercial loans) (the "Maximum Rate"). If a court of competent jurisdiction shall finally determine that Borrower has actually paid to the Lenders an amount of interest in excess of the amount that would have been payable if all of the Secured Obligations had at all times borne interest at the Maximum Rate, then such excess interest actually paid by Borrower shall be applied as follows: first, to the payment of the Secured Obligations consisting of the outstanding principal; second, after all principal is repaid, to the payment of the Lenders' accrued interest, costs, expenses, professional fees and any other Secured Obligations permitted under this Agreement; and third, after all Secured Obligations are repaid, the excess (if any) shall be refunded to Borrower.

2.4 Default Interest. In the event any payment is not paid on the scheduled payment date, an amount equal to four percent (4%) of the past due amount shall be payable on demand. In addition, upon the occurrence and during the continuation of an Event of Default hereunder, all unpaid Secured Obligations, including principal, interest, compounded interest, and professional fees, shall bear interest at a rate per annum equal to the rate set forth in 2.2(c), plus four percent (4%) per annum. In the event any interest is not paid when due hereunder, delinquent interest shall be added to principal and shall bear interest on interest, compounded at the rate set forth in Section 2.2(c) or Section 2.4, as applicable.

2.5 Prepayment. At its option upon at least seven (7) Business Days prior written notice to Agent (or such shorter notice period as agreed by Agent in its discretion), Borrower may prepay all, but not less than all, of the outstanding Advances by paying the entire principal balance, all accrued and unpaid interest thereon, together with a prepayment charge equal to the following percentage of the Advance amount being prepaid: if such prepayment is made (a) during any of the first twelve (12) months following the Closing Date, 3.0%; (b) after twelve (12) months but prior to twenty four (24) months following the Closing Date, 2.0%; and (c) after twenty four (24) months following the Closing Date, but prior to the Term Loan Maturity Date, 1.0% (each, a "Prepayment Charge"). Borrower agrees that the Prepayment Charge is a reasonable calculation of Lenders' lost profits in view of the difficulties and impracticality of determining actual damages resulting from an early repayment of the Advances. Borrower shall prepay the outstanding amount of all principal and accrued interest through the prepayment date and the Prepayment Charge upon the occurrence of a Change in

Control or any other prepayment hereunder. Notwithstanding the foregoing, Agent and Lenders agree to waive the Prepayment Charge if Agent and Lenders (in their sole and absolute discretion) agree in writing to refinance the Advances prior to the Term Loan Maturity Date.

2.6 End of Term Charges.

(a) On the Closing Date, Borrower shall pay Lender a charge in an amount equal to One Million Three Hundred Thousand Dollars ($1,300,000) in connection with the Original A&R LSA Term Loans ("End of Term Charge I"). Notwithstanding the required payment date of the End of Term Charge I, the applicable pro rata portion of the End of Term Charge I shall be deemed earned by each Lender as of each date the applicable Original A&R LSA Term Loan Advances were made. Each Lender acknowledges that End of Term Charge I has been paid in full as of the First Amendment Effective Date.

(b) On the earliest to occur of (i) July 1, 2023, (ii) the date that Borrower prepays the outstanding Secured Obligations (other than any inchoate indemnity obligations and any other obligations which, by their terms, are to survive the termination of this Agreement) in full and (iii) the date that the outstanding Secured Obligations become due and payable, Borrower shall pay Lender a charge in an amount equal to Seventy Hundred Sixty-Three Thousand Seven Hundred Fifty Dollars ($763,750) ("End of Term Charge II") in connection with certain of the Original A&R LSA Term Loans. Notwithstanding the required payment date of the End of Term Charge II, the applicable pro rata portion of the End of Term Charge II shall be deemed earned by each Lender as of each date a Term Loan Advance was made. Each Lender acknowledges that End of Term Charge II has been paid in full as of the First Amendment Effective Date.

(c) On the earliest to occur of (i) the Term Loan Maturity Date, (ii) the date that Borrower prepays the outstanding Secured Obligations (other than any inchoate indemnity obligations and any other obligations which,

by their terms, are to survive the termination of this Agreement) in full ~~and~~, (iii) the date that the outstanding Secured Obligations become due and payable, or (iv) as required pursuant to Section 2.5, Borrower shall pay Lender a charge in the amount equal to (x) One Million Three Hundred Thousand Dollars ($1,300,000), *plus* (y) the product of (A) three and one-half of one percent (3.5%), *multiplied* by (B) the difference of (1) the aggregate principal amount of Term Loan Advances made as of such date, *minus* (2) Thirty-Two Million Five Hundred Thousand Dollars ($32,500,000) ("End of Term Charge III" and together with "End of Term Charge I" and "End of Term Charge II", collectively, the "End of Term Charge"). Notwithstanding the required payment date of the End of Term Charge III, the applicable pro rata portion of the End of Term Charge III shall be deemed earned by each Lender as of each date a Term Loan Advance is made. For the avoidance of doubt, if payment hereunder becomes due and payable on a day that is not a Business Day, the due date thereof shall be the immediately preceding Business Day.

2.7 Notes. If so requested by each Lender by written notice to Borrower, then Borrower shall execute and deliver to Lender (and/or, if applicable and if so specified in such notice, to any Person who is an assignee of Lender pursuant to Section 11.13) (promptly after the Borrower's receipt of such notice) a Note or Notes to evidence Lender's Loans.

2.8 Pro Rata Treatment. Each payment (including prepayment) on account of any fee and any reduction of the Term ~~Loans~~Loan Advances shall be made pro rata according to the Term Commitments of the relevant Lender.

2.9 Treatment of Prepayment Charge and End of Term Charge. Borrower agrees that any Prepayment Charge and any End of Term Charge payable shall be presumed to be the liquidated damages sustained by each Lender as the result of the early termination, and Borrower agrees that it is reasonable under the circumstances currently existing and existing as of the Closing Date and the First Amendment Effective Date. The Prepayment Charge and the End of Term Charge shall also be payable in the event the Secured Obligations (and/or this Agreement) are satisfied or released by foreclosure (whether by power of judicial proceeding), deed in lieu of foreclosure, or by any other means. Borrower expressly waives (to the fullest extent it may lawfully do so) the provisions of any present or future statute or law that prohibits or may prohibit the collection of the foregoing Prepayment Charge and End of Term Charge in connection with any such acceleration. Borrower agrees (to the fullest extent that each may lawfully do so): (a) each of the Prepayment Charge and the End of Term Charge is reasonable and is the product of an arm's length transaction between sophisticated business people, ably represented by counsel; (b) each of the Prepayment Charge and the End of Term Charge shall be payable notwithstanding the then prevailing market rates at the time payment is made; (c) there has been a course of conduct between the Lenders and Borrower giving specific consideration in this transaction for such agreement to pay the Prepayment Charge and the End of Term Charge as a charge (and not interest) in the event of prepayment or acceleration; and (d) Borrower shall be estopped from claiming differently than as agreed to in this paragraph. Borrower expressly acknowledges that their agreement to pay each of the Prepayment Charge and the End of Term Charge to the Lenders as herein described was on the Closing Date and the First Amendment Effective Date and continues to be a material inducement to the Lenders to provide the Term ~~Loans~~Loan Advances.

2.10 Taxes; Increased Costs. The Borrower, the Agent and the Lenders each hereby agree to the terms and conditions set forth on Addendum 1 attached hereto.

SECTION 3. SECURITY INTEREST

3.1 As security for the prompt and complete payment when due (whether on the payment dates or otherwise) of all the Secured Obligations, Borrower grants to Agent a security interest in all of Borrower's right, title, and interest in, to and under all of Borrower's personal property and other assets including without limitation the following (except as set forth herein) whether now owned or hereafter acquired (collectively, the "Collateral"): (a) Receivables; (b) Equipment; (c) Fixtures; (d) General Intangibles (other than Intellectual Property); (e) Inventory; (f) Investment Property; (g) Deposit Accounts; (h) Cash; (i) Goods; and all other tangible and intangible personal property of Borrower whether now or hereafter owned or existing, leased, consigned by or to, or acquired by, Borrower and wherever located, and any of Borrower's property in the possession or under the control of Agent; and, to the extent not otherwise included, all Proceeds of each of the foregoing and all accessions to, substitutions and replacements for, and rents, profits and products of each of the foregoing; provided, however,that theCollateralshall include all Accounts and General Intangibles that consist of rights to payment and proceeds fromthe sale, licensing ordisposition of all or any part, or rights in, the Intellectual Property (the "Rights to Payment"). Notwithstanding the foregoing, if a judicial authority (including a U.S. Bankruptcy Court) holds that a security interest in the underlying Intellectual Property is necessary to have a security interest in the Rights to Payment, then the Collateral shall automatically, and effective as of the date of this Agreement, include the Intellectual Property to the extent necessary to permit perfection of Agent's security interest in the Rights to Payment.

3.2 Notwithstanding the broad grant of the security interest set forth in Section 3.1, above, the Collateral shall not include (a) more than 65% of the presently existing and

hereafter arising issued and outstanding shares of capital stock owned by Borrower of any Foreign Subsidiary (other than an Eligible Foreign Subsidiary) which shares entitle the holder thereof to vote for directors or any other matter, (b) any Intellectual Property except to the extent described in Section 3.1 above, (c) nonassignable licenses or contracts, which by their terms require the consent of the licensor thereof or another party (but only to the extent such prohibition on transfer is enforceable under applicable law, including, without limitation, Sections 9406, 9407 and 9408 of the UCC), and (d) any leasehold real property interest, license, lease or other contract or agreement or any property subject to a purchase money security interest or similar arrangement to the extent that a grant of a security interest therein would violate or invalidate such lease, license, contract or agreement or purchase money arrangement or create a right of termination in favor of any other party thereto (but only to the extent such prohibition on transfer or grant of a security interest is enforceable under applicable law, including, without limitation, Sections 9406, 9407 and 9408 of the UCC), (e) any property to the extent that, and for as long as, such grant of a security interest is prohibited by any applicable law, rule or regulation; provided that the foregoing exclusion in this clause (e) shall in no way be construed (i) to apply to the extent that any described prohibition is unenforceable under Section 9406, 9407 or 9408 of the UCC or other applicable law or (ii) to apply to the extent that any consent or waiver has been obtained, or is hereafter obtained, that would permit the Agent's security interest or Lien notwithstanding the prohibition on the grant of a security interest in such property (f) Excluded Accounts, (g) motor vehicles or other assets in which a security interest may be perfected only though compliance with a certificate of title statute, (h) any property subject to the Sanofi Agreement as disclosed on Schedule 3.2 hereto, and (i) any Cash securing reimbursement obligations permitted under this Agreement.

SECTION 4. CONDITIONS PRECEDENT TO LOAN

The obligations of the Lenders to make the Loan hereunder are subject to the satisfaction by Borrower of the following conditions:

4.1 Initial Advance. On or prior to the Closing Date, Borrower shall have delivered to Agent the following:

(a) executed copies of the Loan Documents, Account Control Agreements, a legal opinion of Borrower's counsel, and all other documents and instruments reasonably required by Agent to effectuate the transactions contemplated hereby or to create and perfect the Liens of Agent with respect to all Collateral, in all cases in form and substance reasonably acceptable to Agent;

(b) certified copy of resolutions of Borrower's board of directors evidencing approval of the Loan and other transactions evidenced by the Loan Documents;

(c) certified copies of the Certificate of Incorporation and the Bylaws, as amended through the Closing Date, of Borrower;

(d) a certificate of good standing for Borrower from its state of incorporation and similar certificates from all other jurisdictions in which it does business and where the failure to be qualified could have a Material Adverse Effect;

(e) payment of the Closing Date Facility Charge, the End of Term Charge I and reimbursement of Agent's and the Lenders' reasonable and documented expenses reimbursable pursuant to this Agreement, which amounts may be deducted from the initial Advance;

(f) all certificates of insurance and to the extent requested by Agent, copies of each insurance policy required hereunder; and

(g) such other documents as Agent may reasonably request.

4.2 All Advances. On each Advance Date:

(a) Agent shall have received (i) an Advance Request for the relevant Advance as required by Section 2.2(b), each duly executed by Borrower's Chief Executive Officer or Chief Financial Officer, and (ii) ~~payment of the applicable Facility Charge then due, and (iii)~~ any other documents Agent may reasonably request.

(b) The representations and warranties set forth in this Agreement shall be true and correct in all material respects on and as of the Advance Date with the same effect as though made on and as of such date, except to the extent such representations and warranties expressly relate to an earlier date, and representations and warranties that may be updated pursuant to this Agreement shall be true and correct in all material respects as of the date made, provided that such updated representations and warranties shall not apply to an earlier date and shall not cure any default arising from any false or incorrect representations and warranties previously made.

(c) Borrower shall be in compliance with all the terms and provisions set forth herein and in each other Loan Document on its part to be observed or performed, and at the time of and immediately after such Advance no Event of Default shall have occurred and be continuing.

(d) Each Advance Request shall be deemed to constitute a representation and warranty by Borrower on the relevant Advance Date as to the matters specified in paragraphs (b) and (c) of this Section 4.2 and as to the matters set forth in the Advance Request.

(e)With respect to any Tranche 6 Advance made available on such Advance Date, the Loan Parties shall have paid the Tranche 6 Facility Charge applicable to such Tranche 6 Advance.

4.3 No Default. As of the Closing Date and each Advance Date, (i) no fact or condition exists that could reasonably (or could reasonably, with the passage of time, the giving of notice, or both) constitute an Event of Default and (ii) no event that has had or could reasonably be expected to have a Material Adverse Effect has occurred and is continuing.

SECTION 5. REPRESENTATIONS AND WARRANTIES OF BORROWER

Borrower represents and warrants that:

5.1 Corporate Status. Borrower is a corporation duly organized, legally existing and in good standing under the laws of the State of Delaware, and is duly qualified as a foreign corporation in all jurisdictions in which the nature of its business or location of its properties require such qualifications and where the failure to be qualified could reasonably be expected to have a Material Adverse Effect. Borrower's present name, former names (if any), locations, place of formation, tax identification number, organizational identification number and other information are correctly set forth in

Exhibit C, as may be updated by Borrower in a written notice (including any Compliance Certificate) provided to Agent after the Closing Date.

5.2 Collateral. Borrower owns the Collateral and the Intellectual Property, free of all Liens, except for Permitted Liens. Borrower has the power and authority to grant to Agent a Lien in the Collateral as security for the Secured Obligations.

5.3 Consents. Borrower's execution, delivery and performance of this Agreement and all other Loan Documents (i) have been duly authorized by all necessary corporate action of Borrower, (ii) will not result in the creation or imposition of any Lien upon the Collateral, other than Permitted Liens and the Liens created by this Agreement and the other Loan Documents, (iii) do not violate any provisions of Borrower's Certificate or Articles of Incorporation (as applicable), bylaws, or any material law, regulation, order, injunction, judgment, decree or writ to which Borrower is subject and (iv) except as described on Schedule 5.3, do not violate any material contract or agreement or require the consent or approval of any other Person which has not already been obtained. The individual or individuals executing the Loan Documents are duly authorized to do so.

5.4 Material Adverse Effect. No event that has had or could reasonably be expected to have a Material Adverse Effect has occurred and is continuing. Borrower is not aware of any event likely to occur that is reasonably expected to result in a Material Adverse Effect.

5.5 Actions Before Governmental Authorities. There are no actions, suits or proceedings at law or in equity or by or before any governmental authority now pending or, to the knowledge of Borrower, threatened in writing against Borrower or its property, that is reasonably expected to result in a Material Adverse Effect.

5.6 Laws. Neither Borrower nor any of its Subsidiaries is in violation of any law, rule or regulation, or in default with respect to any judgment, writ, injunction or decree of any governmental authority, where such violation or default is reasonably expected to result in a Material Adverse Effect. Borrower is not in default in any manner under any provision of any agreement or instrument evidencing material Indebtedness, or any other material agreement to which it is a party or by which it is bound.

Neither Borrower nor any of its Subsidiaries is an "investment company" or a company "controlled" by an "investment company" under the Investment Company Act of 1940, as amended. Neither Borrower nor any of its Subsidiaries is engaged as one of its important activities in extending credit for margin stock (under Regulations X, T and U of the Federal Reserve Board of Governors). Borrower and each of its Subsidiaries has complied in all material respects with the Federal Fair Labor Standards Act. Neither Borrower nor any of its Subsidiaries is a "holding company" or an "affiliate" of a "holding company" or a "subsidiary company" of a "holding company" as each term is defined and used in the Public Utility Holding Company Act of 2005. Neither Borrower's nor any of its Subsidiaries' properties or assets has been used by Borrower or such Subsidiary or, to Borrower's knowledge, by previous Persons, in disposing, producing, storing, treating, or transporting any hazardous substance other than in material compliance with applicable laws. Borrower and each of its Subsidiaries has obtained all consents, approvals and authorizations of, made all declarations or filings with, and given all notices to, all Governmental Authorities that are necessary to continue their respective businesses as currently conducted.

None of Borrower, any of its Subsidiaries, or any of Borrower's or its Subsidiaries' Affiliates or any of their respective agents acting or benefiting in any capacity in connection with the transactions contemplated by this Agreement is (i) in violation of any Anti-Terrorism Law, (ii) engaging in or conspiring to engage in any transaction that evades or avoids, or has the purpose of evading or avoiding or attempts to violate, any of the prohibitions set forth in any Anti-Terrorism Law, or (iii) is a Blocked Person. None of Borrower, any of its Subsidiaries, or to the knowledge of Borrower and any of their Affiliates or agents, acting or benefiting in any capacity in connection with the transactions contemplated by this Agreement, (x) conducts any business or engages in making or receiving any contribution of funds, goods or services to or for the benefit of any Blocked Person, or (y) deals in, or otherwise engages in any transaction relating to, any property or interest in property blocked pursuant to Executive Order No. 13224, any similar executive order or other Anti-Terrorism Law. None of the funds to be provided under this Agreement will be used, directly or indirectly, (a) for any activities in violation of any applicable anti-money laundering, economic sanctions and anti-bribery laws and regulations laws and regulations or (b) for any payment to any governmental official or employee, political party, official of a political party, candidate for political office, or anyone else acting in an official capacity, in order to obtain, retain or direct business or obtain any improper advantage, in violation of the United States Foreign Corrupt Practices Act of 1977, as amended.

5.7 Information Correct and Current. No written information, report, Advance Request, financial statement, exhibit or schedule furnished, by or on behalf of Borrower to Agent in connection with any Loan Document or included therein or delivered pursuant thereto contained, or, when taken as a whole, contains or will contain any material misstatement of fact or, when taken together with all other such information or documents, omitted, omits or will omit to state any material fact necessary to make the statements therein, in the light of the circumstances under which they were, are or will be made, not materially misleading at the time such statement was made or deemed made. Additionally, any and all financial or business projections provided by Borrower to Agent, whether prior to or after the Closing Date, shall be (i) provided in good faith and based on the most current data and information available to Borrower, and (ii) the most current of such projections provided to Borrower's

Board of Directors (it being understood that such projections are subject to significant uncertainties and contingencies, many of which are beyond the control of Borrower, that no assurance is given that any particular projections will be realized, and that actual results may differ).

5.8 Tax Matters. Except as described on Schedule 5.8 ~~and~~, which may be updated by Borrower in a written notice from time to time, and except those being contested in good faith with adequate reserves under GAAP, (a) Borrower has filed all material federal, state and local tax returns that it is required to file, (b) Borrower has duly paid or fully reserved for all taxes or installments thereof (including any interest or penalties) as and when due, which have become due pursuant to such returns, and (c) Borrower has paid or fully reserved for any tax assessment received by Borrower for the three (3) years preceding the Closing Date, if any (including any taxes being contested in good faith and by appropriate proceedings), in each case, other than with respect to taxes that do not exceed, individually or in the aggregate, $50,000.

5.9 Intellectual Property Claims. Borrower is the sole owner of, or otherwise has the right to use, the Intellectual Property material to Borrower's business. Except as described on Schedule 5.9, or in the most recently delivered Compliance Certificate in accordance with Section 7.1(d), (i) to the best knowledge of Borrower, each of the material Copyrights, Trademarks and Patents is valid and enforceable, (ii) no material part of the Intellectual Property has been judged invalid or unenforceable, in whole or in part,

and (iii) no claim has been made to Borrower that any material part of the Intellectual Property violates the rights of any third party. Exhibit D (which may be updated by Borrower in a written notice from time to time) is a true, correct and complete list of each of Borrower's Patents, registered Trademarks, registered Copyrights, and material agreements under which Borrower licenses Intellectual Property from third parties (other than shrink-wrap software licenses), together with application or registration numbers, as applicable, owned by Borrower or any Subsidiary, in each case as of the Closing Date. Borrower is not in material breach of, nor has Borrower failed to perform any material obligations under, any of the foregoing contracts, licenses or agreements and, to Borrower's knowledge, no third party to any such contract, license or agreement is in material breach thereof or has failed to perform any material obligations thereunder.

5.10 Intellectual Property. Except as described on Schedule 5.10, which may be updated by Borrower in a written notice from time to time, to the best knowledge of Borrower, Borrower has all material rights with respect to Intellectual Property that it uses in, and that is necessary or material in the operation or conduct of Borrower's business as currently conducted and proposed to be conducted by Borrower. Without limiting the generality of the foregoing, and in the case of Licenses, except for restrictions that are unenforceable under Division 9 of the UCC, Borrower has the right, to the extent required to operate Borrower's business, to freely transfer, license or assign Intellectual Property necessary or material in the operation or conduct of Borrower's business as currently conducted by Borrower, without condition, restriction or payment of any kind (other than license payments in the ordinary course of business) to any third party, and Borrower owns or has the right to use, pursuant to valid licenses, all software development tools, library functions, compilers and all other third-party software and other items that are material to Borrower's business and used in the design, development, promotion, sale, license, manufacture, import, export, use or distribution of Borrower Products that are material to Borrower's business except customary covenants in inbound license agreements and equipment leases where Borrower is the licensee or lessee.

5.11 Borrower Products. Except as described on Schedule 5.11, which may be updated by Borrower in a written notice ~~provided after the Closing Date~~from time to time, no Intellectual Property owned by Borrower or Borrower Product has been or is subject to any actual or, to the knowledge of Borrower, threatened in writing litigation, proceeding (including any proceeding in the United States Patent and Trademark Office or any corresponding foreign office or agency) or outstanding decree, order, judgment, settlement agreement or stipulation that restricts in any manner Borrower's use, transfer or licensing thereof or that may affect the validity, use or enforceability thereof, in such case, which could reasonably be expected to have a Material Adverse Effect. There is no decree, order, judgment, agreement, stipulation, arbitral award or other provision entered into in connection with any litigation or proceeding that obligates Borrower to grant licenses or ownership interest in any future material Intellectual Property related to the operation or conduct of the business of Borrower or Borrower Products. Except as set forth on Schedule 5.11, which may be updated by Borrower in a written notice from time to time, Borrower has not received any written notice or claim, challenging or questioning Borrower's ownership in any Intellectual Property material to Borrower's business (or written notice of any claim challenging or questioning the ownership in any material licensed Intellectual Property of the owner thereof) or suggesting that any third party has any claim of legal or beneficial ownership with respect thereto nor, to Borrower's knowledge, is there a reasonable basis for any such claim. Neither Borrower's use of its Intellectual Property material to Borrower's business nor the production and sale of Borrower Products material to Borrower's business infringes in any material respect the Intellectual Property or other rights of others.

5.12 Financial Accounts. Exhibit E, as may be updated by the Borrower in a written notice provided to Agent after the Closing Date, is a true, correct and complete list of (a) all banks and other financial institutions at which Borrower or any Subsidiary maintains Deposit Accounts and (b) all institutions at which Borrower or any Subsidiary maintains an account holding Investment Property, and such exhibit correctly identifies the name, address and telephone number of each bank or other institution, the name in which the account is held, a description of the purpose of the account, and the complete account number therefor.

5.13 Employee Loans. Except as described on Schedule 5.13, which may be updated by Borrower in a written notice provided after the Closing Date, Borrower has no outstanding loans to any employee, officer or director of the Borrower nor has Borrower guaranteed the payment of any loan made to an employee, officer or director of the Borrower by a third party.

5.14 Capitalization and Subsidiaries. Borrower's capitalization as of the Closing Date is set forth on Schedule 5.14 annexed hereto, which may be updated by Borrower in a written notice from time to time. Borrower does not own any stock, partnership interest or other securities of any Person, except for Permitted Investments. Attached as Schedule 5.14, as may be updated by Borrower in a written notice provided after the Closing Date, is a true, correct and complete list of each Subsidiary.

5.15 Foreign Subsidiary Voting Rights. No decision or action in any governing document of any Foreign Subsidiary (other than an Eligible Foreign Subsidiary) requires a vote of greater than 50.1% of the Equity Interests or voting rights of such Foreign Subsidiary.

SECTION 6. INSURANCE; INDEMNIFICATION

6.1 Coverage. Borrower shall cause to be carried and maintained commercial general liability insurance, on an occurrence form, against risks customarily insured against in Borrower's line of business. Such risks shall

include the risks of bodily injury, including death, property damage, personal injury, advertising injury, and contractual liability per the terms of the indemnification agreement found in Section 6.3. Borrower must maintain a minimum of $2,000,000 of commercial general liability insurance for each occurrence. Borrower has and agrees to maintain a minimum of $2,000,000 of directors' and officers' insurance for each occurrence and $5,000,000 in the aggregate. So long as there are any Secured Obligations outstanding (other than inchoate indemnity obligations and other obligations which are expressly stated to survive termination of this Agreement), Borrower shall also cause to be carried and maintained insurance upon the Collateral, insuring against all risks of physical loss or damage howsoever caused, in an amount not less than the full replacement cost of the Collateral, provided that such insurance may be subject to standard exceptions and deductibles. If Borrower fails to obtain the insurance called for by this Section 6.1 or fails to pay any premium thereon or fails to pay any other amount which Borrower is obligated to pay under this Agreement or any other Loan Document or which may be required to preserve the Collateral, Agent may obtain such insurance or make such payment, and all amounts so paid by Agent are immediately due and payable, bearing interest at the then highest rate applicable to the Secured Obligations, and secured by the Collateral. Agent will make reasonable efforts to provide Borrower with notice of Agent obtaining such insurance at the time it is obtained or within a reasonable time thereafter. No payments by Agent are deemed an agreement to make similar payments in the future or Agent's waiver of any Event of Default.

6.2 Certificates. Borrower shall deliver to Agent certificates of insurance that evidence Borrower's compliance with its insurance obligations in Section 6.1 and the obligations contained in this Section 6.2. Borrower's insurance certificate shall state Agent (shown as "Hercules Capital, Inc.", as Agent, and its successors and/or assigns") is an additional insured for commercial general liability, a loss payee for all risk property damage insurance, subject to the insurer's approval, and a loss payee for property insurance and additional insured for liability insurance for any future insurance that Borrower may acquire from such insurer. Attached to the certificates of insurance will be additional insured endorsements for liability and lender's loss payable endorsements for all risk property damage insurance. All certificates of insurance will provide for a minimum of thirty (30) days advance written notice to Agent of cancellation (other than cancellation for non-payment of premiums, for which ten (10) days' advance written notice shall be sufficient) or any other change adverse to Agent's interests. Any failure of Agent to scrutinize such insurance certificates for compliance is not a waiver of any of Agent's rights, all of which are reserved. Borrower shall provide Agent with copies of each insurance policy within thirty (30) days of the Closing Date, and upon entering or amending any insurance policy required hereunder, Borrower shall provide Agent with copies of such policies and shall promptly deliver to Agent updated insurance certificates with respect to such policies.

6.3 Indemnity. Borrower agrees to indemnify and hold Agent, the Lenders and their officers, directors, employees, agents, in-house attorneys, representatives and shareholders (each, an "Indemnified Person") harmless from and against any and all claims, costs, expenses, damages and liabilities (including such claims, costs, expenses, damages and liabilities based on liability in tort, including strict liability in tort), including reasonable attorneys' fees and disbursements and other costs of investigation or defense (including those incurred upon any appeal) (collectively, "Liabilities"), that may be instituted or asserted against or incurred by such Indemnified Person as the result of credit having been extended, suspended or terminated under this Agreement and the other Loan Documents or the administration of such credit, or in connection with or arising out of the transactions contemplated hereunder and thereunder, or any actions or failures to act in connection therewith, or arising out of the disposition or utilization of the Collateral, excluding in all cases Liabilities to the

extent resulting solely from any Indemnified Person's gross negligence or willful misconduct. Borrower agrees to pay, and to save Agent and Lender harmless from, any and all liabilities with respect to, or resulting from any delay in paying, any and all excise, sales or other similar taxes (excluding taxes imposed on or measured by the net income of Agent or Lender) that may be payable or determined to be payable with respect to any of the Collateral or this Agreement, excluding in all cases Liabilities to the extent resulting directly from any Indemnified Person's gross negligence or willful misconduct. In no event shall any Indemnified Person be liable on any theory of liability for any special, indirect, consequential or punitive damages (including any loss of profits, business or anticipated savings). This Section 6.3 shall not apply with respect to taxes other than any taxes that represent losses, claims, damages, etc. arising from any non-tax claim. This Section 6.3 shall survive the repayment of indebtedness under, and otherwise shall survive the expiration or other termination of, the Agreement.

SECTION 7. COVENANTS OF BORROWER

Borrower agrees as follows:

7.1 Financial Reports. Borrower shall furnish to Agent the financial statements and reports listed hereinafter (the "Financial Statements"):

(a) as soon as practicable (and in any event within 30 days) after the end of each month, unaudited interim and year-to-date financial statements as of the end of such month (prepared on a consolidated and consolidating basis, if applicable), including balance sheet and related statements of income and cash flows accompanied by a report detailing any material contingencies (including the commencement of any material litigation by or against Borrower) or any other occurrence that could reasonably be expected to have a Material Adverse Effect, all certified by Borrower's Chief Executive Officer or Chief Financial Officer to the effect that they have been prepared in accordance with GAAP, except (i) for the absence of footnotes, (ii) that they are subject to normal year-end adjustments, and (iii) they do not contain certain non-cash items that are customarily included in quarterly and annual financial statements;

(b) as soon as practicable (and in any event within 45 days) after the end of each of the first three calendar quarters of each year, unaudited interim and year-to-date financial statements as of the end of such calendar quarter (prepared on a consolidated and consolidating basis, if applicable), including balance sheet and related statements of income and cash flows accompanied by a report detailing any material contingencies (including the commencement of any material litigation by or against Borrower) or any other occurrence that could reasonably be expected to have a Material Adverse Effect, certified by Borrower's Chief Executive Officer or Chief Financial Officer to the effect that they have been prepared in accordance with GAAP, except (i) for the absence of footnotes, and (ii) that they are subject to normal year-end adjustments;

(c) as soon as practicable (and in any event within ninety (90) days) after the end of each fiscal year, unqualified audited financial statements as of the end of such year (prepared on a consolidated and consolidating basis, if applicable), including balance sheet and related statements of income and cash flows, and setting forth in comparative form the corresponding figures for the preceding fiscal year, certified by a firm of independent certified public accountants selected by Borrower and reasonably acceptable to Agent, accompanied by any management report from such accountants (Agent hereby acknowledges that PwC is an acceptable firm of independent public accountants);

(d) as soon as practicable (and in any event within 30 days) after the end of each month, a Compliance Certificate in the form of Exhibit F;

(e) as soon as practicable (and in any event within 30 days) after the end of each month, a report showing agings of accounts receivable and accounts payable;

(f) promptly and in any event within 5 days after the sending or filing thereof, as the case may be, copies of any proxy statements, financial statements or reports that Borrower has made generally available to holders of its Common Stock and copies of any regular, periodic and special reports or registration statements that Borrower files with the Securities and Exchange Commission or any governmental authority that may be substituted therefor, or any national securities exchange;

(g) at the same time and in the same manner as it gives to its Board of Directors, copies of all board packages that Borrower provides to its directors in connection with meetings of the Board of Directors, and within 30 days after each such meeting, minutes of such meeting, provided that in all cases Borrower may exclude confidential compensation information, information presenting a conflict of interest with Agent or Lender and information covered by attorney-client privilege;

(h) any budget and any forecast of Borrower promptly following its approval by Borrower's Board of Directors, and in any event, not less than once annually, no later than 60 days following the end of the fiscal year;

(i) such other financial information reasonably requested by Agent; and

(j) immediate notice if Borrower or any Subsidiary has knowledge that Borrower, or any Subsidiary or Affiliate of Borrower, is listed on the OFAC Lists or (a) is convicted on, (b) pleads *nolo contendere* to, (c) is indicted on, or (d) is arraigned and held over on charges involving money laundering or predicate crimes to money laundering.

Borrower shall not (without the consent of Agent, such consent not to be unreasonably withheld or delayed), make any change in its (a) accounting policies or reporting practices, except as required by GAAP or (b) fiscal years or fiscal quarters. As of the Closing Date, the fiscal year of Borrower ends on December 31.

The executed Compliance Certificate and all Financial Statements required to be delivered pursuant to clauses (a), (b) and (c) shall be sent via e-mail to with a copy to , , and l and provided, that if e-mail is not available or sending such Financial Statements via e-mail is not possible, they shall be faxed to Agent at: , attention Account Manager: X4 Pharmaceuticals, Inc.

Notwithstanding the foregoing, documents required to be delivered pursuant to the terms hereof (to the extent any such documents are included in materials otherwise filed with the SEC) may be delivered electronically and if so delivered, shall be deemed to have been delivered on the date on which Borrower emails a link thereto to Agent.

7.2 Management Rights. Borrower shall permit any representative that Agent or the Lenders authorize, including its attorneys and accountants, to inspect the Collateral and examine and make copies and abstracts of the books of account and records of Borrower at reasonable times and upon reasonable notice during normal business hours; provided, however, that so long as no Event of Default has occurred and is continuing, such examinations shall be limited to no more often than twice per fiscal year. In addition, any such representative shall

have the right to meet with management and officers of Borrower to discuss such books of account and records at reasonable times and upon reasonable notice during normal business hours. In addition, Agent or Lender shall be entitled at reasonable times and intervals to consult with and advise the management and officers of Borrower concerning significant business issues affecting Borrower; provided that management and officers of Borrower shall not be bound to accept such advisement. Such consultations shall not unreasonably interfere with Borrower's business operations. The parties intend that the rights granted Agent and the Lenders shall constitute "management rights" within the meaning of 29 C.F.R. Section 2510.3-101(d)(3)(ii), but that any advice, recommendations or participation by Agent or the Lenders with respect to any business issues shall not be deemed to give Agent or the Lenders, nor be deemed an exercise by Agent or the Lenders of, control over Borrower's management or policies.

7.3 Further Assurances. Borrower shall from time to time following Agent's request execute, deliver and file, alone or with Agent, any financing statements, security agreements, collateral assignments, notices, control agreements, or other documents to perfect or give the highest priority to Agent's Lien on the Collateral or otherwise evidence Agent's rights herein. Borrower shall from time to time procure any instruments or documents as may be reasonably requested by Agent, and take all further action that may be necessary, or that Agent may reasonably request, to perfect and protect the Liens granted hereby and thereby. In addition, and for such purposes only, Borrower hereby authorizes Agent to execute and deliver on behalf of Borrower and to file such financing statements (including an indication that the financing statement covers "all assets or all personal property" of Borrower in accordance with Section 9-504 of the UCC), collateral assignments, notices, control agreements, security agreements and other documents without the signature of Borrower either in Agent's name or in the name of Agent as agent and attorney-in-fact for Borrower. Borrower shall protect and defend Borrower's title to the Collateral and Agent's Lien thereon against all Persons claiming any interest adverse to Borrower or Agent other than Permitted Liens.

7.4 Indebtedness. Borrower shall not create, incur, assume, guarantee or be or remain liable with respect to any Indebtedness, or permit any Subsidiary so to do, other than Permitted Indebtedness, or prepay any Indebtedness or take any actions which impose on Borrower an obligation to prepay any Indebtedness, except for (a) the conversion of Indebtedness into equity securities and the payment of cash in lieu of fractional shares in connection with such conversion, (b) purchase money Indebtedness pursuant to its then applicable payment schedule, (c) prepayment by any Subsidiary of (i) inter-company Indebtedness owed by such Subsidiary to any Borrower, or (ii) if such Subsidiary is not a Borrower, intercompany Indebtedness owed by such Subsidiary to another Subsidiary that is not a Borrower, or (d) as otherwise permitted hereunder or approved in writing by Agent.

7.5 Collateral. Borrower shall at all times keep the Collateral, the Intellectual Property and all other property and assets used in Borrower's business or in which Borrower now or hereafter holds any interest free and clear from any legal process or Liens whatsoever (except for Permitted Liens), and shall give Agent prompt written notice of any legal process affecting the Collateral, the Intellectual Property, such other property and assets, or any Liens thereon, provided however, that the Collateral and such other property and assets may be subject to Permitted Liens except that there shall be no Liens whatsoever on Intellectual Property other than (i) customary restrictions on assignment, sublicense or transfer that may exist in any license agreement where Borrower or a Subsidiary is the licensee (and not the licensor) and (ii) licenses of Intellectual Property that constitute Permitted Transfers. Borrower shall not agree with any Person other than Agent or Lender not to encumber its property other than pursuant to customary restrictions in leases, licenses and agreements in respect

of Permitted Indebtedness. Borrower shall not enter into or suffer to exist or become effective any agreement that prohibits or limits the ability of any Borrower to create, incur, assume or suffer to exist any Lien upon any of its Intellectual Property, whether now owned or hereafter acquired, to secure its obligations under the Loan Documents to which it is a party other than (a) this Agreement and the other Loan Documents, (b) any agreements governing any purchase money Liens or capital lease obligations otherwise permitted hereby (in which case, any prohibition or limitation shall only be effective against the assets financed thereby) and (c) customary restrictions on the assignment of leases, licenses and other agreements. Borrower shall cause its Subsidiaries to protect and defend such Subsidiary's title to its assets from and against all Persons claiming any interest adverse to such Subsidiary, and Borrower shall cause its Subsidiaries at all times to keep such Subsidiary's property and assets free and clear from any legal process or Liens whatsoever (except for Permitted Liens, provided however, that there shall be no Liens whatsoever on Intellectual Property other than (i) customary restrictions on assignment, sublicense or transfer that may exist in any license agreement where Borrower or a Subsidiary is the licensee (and not the licensor) and (ii) licenses of Intellectual Property that constitute Permitted Transfers), and shall give Agent prompt written notice of any legal process affecting such Subsidiary's assets.

7.6 Investments. Borrower shall not directly or indirectly acquire or own, or make any Investment in or to any Person, or permit any of its Subsidiaries to do so, other than Permitted Investments.

7.7 Distributions. Borrower shall not, and shall not allow any Subsidiary to, (a) repurchase or redeem any class of stock or other Equity Interest other than pursuant to employee, director or consultant repurchase plans or other similar agreements, provided, however, in each case the repurchase or redemption price does not exceed the original consideration paid for such stock or Equity Interest, or (b) declare or pay any cash dividend or make a cash distribution on any class of stock or other Equity Interest, except that a Subsidiary may pay dividends or make distributions to Borrower, or (c) lend money to any employees, officers or directors (except for Permitted Investments) or guarantee the payment of any such loans granted by a third party in excess of $250,000 in the aggregate or (d) waive, release or forgive any Indebtedness owed by any employees, officers or directors in excess of $250,000 in the aggregate.

7.8 Transfers. Except for Permitted Transfers, Borrower shall not, and shall not allow any Subsidiary to, voluntarily or involuntarily transfer, sell, lease, license, lend or in any other manner convey any equitable, beneficial or legal interest in any material portion of its assets.

7.9 Mergers or Acquisitions. Borrower shall not merge or consolidate, or permit any of its Subsidiaries to merge or consolidate, with or into any other business organization (other than mergers or consolidations of (a) a Subsidiary which is not a Borrower into another Subsidiary or into Borrower or (b) a Borrower into another Borrower), or acquire, or permit any of its Subsidiaries to acquire, in each case including for the avoidance of doubt through a merger, purchase, in-licensing arrangement or any similar transaction, all or substantially all of the capital stock or any property of another Person, other than in connection with a Permitted Investment or Permitted Acquisition.

7.10 Taxes. Borrower and its Subsidiaries shall pay when due all material taxes, fees or other charges of any nature whatsoever (together with any related interest or penalties) now or hereafter imposed or assessed against Borrower, Agent, Lender or the Collateral or upon Borrower's ownership, possession, use, operation or disposition thereof or upon Borrower's rents, receipts or earnings arising therefrom. Borrower shall file on or before the due date therefor all personal property tax returns in respect of the Collateral. Notwithstanding the

foregoing, Borrower may contest, in good faith and by appropriate proceedings, taxes for which Borrower maintains adequate reserves therefor in accordance with GAAP.

7.11 Corporate Changes. Neither Borrower nor any Subsidiary shall change its corporate name, legal form or jurisdiction of formation without twenty (20) days' prior written notice to Agent. Neither Borrower nor any Subsidiary shall suffer a Change in Control. Neither Borrower nor any Subsidiary shall relocate its chief executive office or its principal place of business unless: (i) it has provided prior written notice to Agent; and (ii) such relocation shall be within the continental United States of America. Neither Borrower nor any Qualified Subsidiary shall relocate any item of Collateral (other than (x) sales of Inventory in the ordinary course of business, (y) relocations of Equipment having an aggregate value of up to $250,000 in any fiscal year, and (z) relocations of Collateral from a location described on Exhibit C to another location described on Exhibit C) unless (i) it has provided prompt written notice to Agent, (ii) such relocation is within the continental United States of America and, (iii) if such relocation is to a third party bailee, it has delivered a bailee agreement in form and substance reasonably acceptable to Agent.

7.12 Deposit Accounts. Neither Borrower nor any Qualified Subsidiary shall maintain any Deposit Accounts, or accounts holding Investment Property, except (i) for Excluded Accounts, or (ii) with respect to which Agent has an Account Control Agreement. Notwithstanding anything to the contrary in this Agreement, the Excluded Subsidiary may maintain Deposit Accounts which are not subject to an Account Control Agreement, provided that the aggregate amount in such Deposit Accounts does not at any time exceed $5,000,000, provided, however, that Borrower at all times shall be in compliance with the provisions of Section 7.21 below.

7.13 Notification of New Subsidiaries. Borrower shall notify Agent of each Subsidiary formed subsequent to the Closing Date and, within 15 days of formation, shall cause any such Qualified Subsidiary to execute and deliver to Agent a Joinder Agreement.

7.14 [Reserved].

7.15 Notification of Event of Default. Borrower shall notify Agent immediately of the occurrence of any Event of Default.

7.16 Use of Proceeds. Borrower agrees that the proceeds of the Loans shall be used solely to refinance existing indebtedness, to pay related fees and expenses in connection with this Agreement and for working capital and general corporate purposes, including strategic licensing and acquisition opportunities permitted under this Agreement or consented to by Agent. The proceeds of the Loans will not be used in violation of Anti-Corruption Laws or applicable Sanctions.

7.17 Foreign Subsidiary Voting Rights. Borrower shall not, and shall not permit any Subsidiary, to amend or modify any governing document of any Foreign Subsidiary of Borrower (other than an Eligible Foreign Subsidiary) the effect of which is to require a vote of greater than 50.1% of the Equity Interests or voting rights of such entity for any decision or action of such entity.

7.18 Compliance with Laws.

(a) Borrower shall maintain, and shall cause its Subsidiaries to maintain, compliance in all material respects with all applicable laws, rules or regulations (including any law, rule or regulation with respect to the

making or brokering of loans or financial accommodations), and shall, or cause its Subsidiaries to, obtain and maintain all required governmental authorizations, approvals, licenses, franchises, permits or registrations reasonably necessary in connection with the conduct of Borrower's business.

(b) Neither Borrower nor any of its Subsidiaries shall, nor shall Borrower or any of its Subsidiaries permit any Affiliate controlled by Borrower or any Subsidiary to, directly or indirectly, knowingly enter into any documents, instruments, agreements or contracts with any Person listed on the OFAC Lists. Neither Borrower nor any of its Subsidiaries shall, nor shall Borrower or any of its Subsidiaries, permit any Affiliate controlled by Borrower or any Subsidiary to, directly or indirectly, (i) conduct any business or engage in any transaction or dealing with any Blocked Person, including, without limitation, the making or receiving of any contribution of funds, goods or services to or for the benefit of any Blocked Person, (ii) deal in, or otherwise engage in any transaction relating to, any property or interests in property blocked pursuant to Executive Order No. 13224 or any similar executive order or other Anti-Terrorism Law, or (iii) engage in or conspire to engage in any transaction that evades or avoids, or has the purpose of evading or avoiding, or attempts to violate, any of the prohibitions set forth in Executive Order No. 13224 or other Anti-Terrorism Law.

(c) Borrower has implemented and maintains in effect policies and procedures designed to ensure compliance by the Borrower, its Subsidiaries and their respective directors, officers, employees and agents with Anti-Corruption Laws and applicable Sanctions, and Borrower and its Subsidiaries and their respective officers and employees and to the knowledge of Borrower, its directors and agents, are in compliance with Anti-Corruption Laws and applicable Sanctions in all material respects.

(d) None of Borrower, any of its Subsidiaries or any of their respective directors, officers or employees, or to the knowledge of Borrower, any agent for Borrower or its Subsidiaries that will act in any capacity in connection with or benefit from the credit facility established hereby, is a Sanctioned Person. No Loan, use of proceeds or other transaction contemplated by this Agreement will violate Anti-Corruption Laws or applicable Sanctions.

7.19 Intellectual Property. Each Borrower shall (i) protect, defend and maintain the validity and enforceability of its Intellectual Property material to its business; (ii) promptly advise Agent in writing of material infringements of its Intellectual Property; and (iii) not allow any Intellectual Property material to Borrowers' business to be abandoned, forfeited or dedicated to the public without Agent's written consent. If a Borrower (i) obtains any Patent, registered Trademark, registered Copyright, registered mask work, or any pending application for any of the foregoing, whether as owner, licensee or otherwise, or (ii) applies for any Patent or the registration of any Trademark, then such Borrower shall promptly, and in any event, within thirty (30) days thereof, provide written notice thereof to Agent. If a Borrower decides to register any Copyrights or mask works in the United States Copyright Office, such Borrower shall provide Agent with at least fifteen (15) days prior written notice of such Borrower's intent to register such Copyrights or mask works together with a copy of the application it intends to file with the United States Copyright Office (excluding exhibits thereto). Borrowers shall promptly provide to Agent copies of all applications that it files for Patents or for the registration of Trademarks, Copyrights or mask works.

7.20 Transactions with Affiliates. Borrower shall not and shall not permit any Subsidiary to, directly or indirectly, enter into or permit to exist any transaction of any kind with any Affiliate of Borrower or such Subsidiary

on terms that are less favorable to Borrower or such Subsidiary, as the case may be, than those that might be obtained in an arm's length transaction from a Person who is not an Affiliate of Borrower or such Subsidiary.

7.21 Minimum Cash.

(a) Prior to January 31, 2025, Borrower shall at all times ~~prior to the Approval Milestone Date,~~ maintain Qualified Cash in an aggregate amount greater than or equal to Twenty Million Dollars ($20,000,000);

(b) ~~Notwithstanding the provisions of Section 7.21(a) above, Borrower shall at~~At all times on and after ~~the Approval Milestone Date,~~January 31, 2025, Borrower shall maintain Qualified Cash in an aggregate amount ~~greater than or equal Ten Million Dollars ($10,000,000)~~equal to at least 20% of the aggregate principal amount of Term Loan Advances outstanding under this Agreement.

(c) Notwithstanding the foregoing, in the event of any Permitted Acquisition, the minimum Cash levels set forth in this Section 7.21 may be reset, subject to ~~Agent's discretion~~mutual agreement between Agent and Borrower.

7.22 Performance Covenant.

(a) Subject to Section 7.22(b), from and after January 31, 2025, Borrower shall maintain T6M Net Product Revenue, tested monthly, of at least 55% of Net Product Revenue Forecast.

(b) The requirement in Section 7.22(a) shall be waived during any period for which either (i) Borrower maintains Qualified Cash in an amount equal to or greater than ~~80~~75% of the aggregate principal amount of Term Loan Advances outstanding under this Agreement or (ii) both (x) Company maintains a Market Capitalization of at least $~~500,000,000~~450,000,000 and (y) Borrower maintains Qualified Cash in an amount equal to at least ~~50~~45% of the aggregate principal amount of Term Loan Advances outstanding under this Agreement (for the avoidance of doubt, this waiver provision is a daily condition and, if it is not satisfied at any point in time, compliance with Section 7.22(a) would need to be demonstrated as of the most recent financial reporting period).

7.23 Security Corporation Investment Conditions. At any time that the Security Corporation Subsidiary has any assets or liabilities, Borrower shall satisfy the Security Corporation Investment Conditions at all times.

7.24 Post-Closing Items. On or before the corresponding dates set forth on Schedule 7.24, Borrower shall use its commercially reasonable efforts to deliver or cause to be delivered or completed the items listed on Schedule 7.24.

SECTION 8. RIGHT TO INVEST

8.1 The Lenders or their assignee or nominee shall have the right, in its discretion, to participate, in a cumulative amount of up to $1,000,000 in the aggregate, in one or more Subsequent Financings, on the same terms, conditions and pricing afforded to others participating in any such Subsequent Financing, provided, however, Lender or its assignee or nominee agrees to become a party to the agreements executed by the others participating in such Subsequent Financing. Notwithstanding the foregoing, the Company shall provide the Lender or its assignee or nominee at least one (1) Business Days' notice (which may be oral) of a planned Subsequent Financing and the opportunity to exercise the right to invest under this Section 8.1 with respect to

such Subsequent Financing. This Section 8.1, and all rights and obligations hereunder, shall terminate upon the earlier to occur of (i) such time that the Lender or its assignees or nominees have purchased $1,000,000 of the Company's equity securities in the aggregate in any Subsequent Financing(s) and (ii) the later to occur of (A) the repayment of the Indebtedness under this Agreement and (B) the exercise in full of the Warrants or the expiration or termination of the exercise period for the Warrants.

SECTION 9. EVENTS OF DEFAULT

The occurrence of any one or more of the following events shall be an "Event of Default":

9.1 Payments. Borrower fails to pay any amount due under this Agreement or any of the other Loan Documents on the due date; provided, however, that an Event of Default shall not occur on account of a failure to pay due solely to an administrative or operational error of Agent or the Lenders or Borrower's bank if Borrower had the funds to make the payment when due and makes the payment within three (3) Business Days following Borrower's knowledge of such failure to pay; or

9.2 Covenants. Borrower breaches or defaults in the performance of any covenant or Secured Obligation under this Agreement, or any of the other Loan Documents or any other agreement among Borrower, Agent and the Lenders, and (a) with respect to a ~~default~~Default under any covenant under this Agreement (other than under Sections 6, 7.4, 7.5, 7.6, 7.7, 7.8, 7.9, 7.15, 7.16, 7.17, 7.18, 7.19, 7.21, 7.22, 7.23 and 7.24) any other Loan Document or any other agreement among Borrower, Agent and the Lenders, such default continues for more than ten (10) Business Days after the earlier of the date on which (i) Agent or the Lenders has given notice of such default to Borrower and (ii) Borrower has actual knowledge of such default or (b) with respect to a ~~default~~Default under any of Sections 6, 7.4, 7.5, 7.6, 7.7, 7.8, 7.9, 7.15, 7.16, 7.17, 7.18, 7.19, 7.21, 7.22, 7.23 and 7.24, the occurrence of such ~~default~~Default; or

9.3 Material Adverse Effect. A circumstance has occurred that could reasonably be expected to have a Material Adverse Effect; provided that, solely for the purposes of this Section 9.3, the failure of the occurrence of the Tranche 5 Milestone Date shall in and of itself not constitute a Material Adverse Effect; or

9.4 Representations. Any representation or warranty made by Borrower in any Loan Document shall have been false or misleading in any material respect when made or when deemed made; or

9.5 Insolvency. Borrower (A) (i) shall make an assignment for the benefit of creditors; or (ii) shall be unable to pay its debts as they become due, or be unable to pay or perform under the Loan Documents, or shall become insolvent; or (iii) shall file a voluntary petition in bankruptcy; or (iv) shall file any petition, answer, or document seeking for itself any reorganization, arrangement, composition, readjustment, liquidation, dissolution or similar relief under any present or future statute, law or regulation pertinent to such circumstances; or (v) shall seek or consent to or acquiesce in the appointment of any trustee, receiver, or liquidator of Borrower or of all or any substantial part (i.e., 33-1/3% or more) of the assets or property of Borrower; or (vi) shall cease operations of its business as its business has normally been conducted, or terminate substantially all of its employees; or (vii) Borrower or its directors or majority shareholders shall take any action initiating any of the foregoing actions described in clauses (i) through (vi); or (B) either (i) forty-five (45) days shall have expired after the commencement of an involuntary action against Borrower seeking reorganization, arrangement, composition, readjustment, liquidation, dissolution or similar relief under any present or future statute, law or regulation, without such action being dismissed or all orders or proceedings thereunder affecting the operations or the business of

Borrower being stayed; or (ii) a stay of any such order or proceedings shall thereafter be set aside and the action setting it aside shall not be timely appealed; or (iii) Borrower shall file any answer admitting or not contesting the material allegations of a petition filed against Borrower in any such proceedings; or (iv) the court in which such proceedings are pending shall enter a decree or order granting the relief sought in any such proceedings; or (v) forty-five (45) days shall have expired after the appointment, without the consent or acquiescence of Borrower, of any trustee, receiver or liquidator of Borrower or of all or any substantial part of the properties of Borrower without such appointment being vacated; or

9.6 Attachments; Judgments. Any portion of Borrower's assets is attached or seized, or a levy is filed against any such assets, or a judgment or judgments is/are entered for the payment of money (not covered by independent third party insurance as to which liability has not been rejected by such insurance carrier), individually or in the aggregate, of at least $500,000, or Borrower is enjoined or in any way prevented by court order from conducting a material part of its business and such attachment, seizure, levy, judgment or enjoinment is not, within thirty (30) days after the occurrence thereof, discharged or stayed (whether through the posting of a bond or otherwise); or

9.7 Other Obligations. The occurrence of any "event of default" under any agreement or obligation of Borrower involving any Indebtedness in excess of $500,000 after giving effect to any applicable grace period thereunder which results in a right by such third party to accelerate the maturity of such Indebtedness.

SECTION 10. REMEDIES

10.1 General. Upon and during the continuance of any one or more Events of Default, (i) Agent may, and at the direction of the Required Lenders shall, accelerate and demand payment of all or any part of the Secured Obligations together with a Prepayment Charge and declare them to be immediately due and payable (provided, that upon the occurrence of an Event of Default of the type described in Section 9.5, all of the Secured Obligations (including, without limitation, the Prepayment Charge and the End of Term Charge) shall automatically be accelerated and made due and payable, in each case without any further notice or act). Borrower hereby irrevocably appoints Agent as its lawful attorney-in-fact to: (a) exercisable following the occurrence and during the continuance of an Event of Default, (i) sign Borrower's name on any invoice or bill of lading for any account or drafts against account debtors; (ii) demand, collect, sue, and give releases to any account debtor for monies due, settle and adjust disputes and claims about the accounts directly with account debtors, and compromise, prosecute, or defend any action, claim, case, or proceeding about any Collateral (including filing a claim or voting a claim in any bankruptcy case in Agent's or Borrower's name, as Agent may elect); (iii) make, settle, and adjust all claims under Borrower's insurance policies; (iv) pay, contest or settle any Lien, charge, encumbrance, security interest, or other claim in or to the Collateral, or any judgment based thereon, or otherwise take any action to terminate or discharge the same; (v) transfer the Collateral into the name of Agent or a third party as the UCC permits; and (vi) receive, open and dispose of mail addressed to Borrower; and (b) regardless of whether an Event of Default has occurred, (i) endorse Borrower's name on any checks, payment instruments, or other forms of payment or security; and (ii) notify all account debtors to pay Agent directly. Borrower hereby appoints Agent as its lawful attorney-in-fact to sign Borrower's name on any documents necessary to perfect or continue the perfection of Agent's security interest in the Collateral regardless of whether an Event of Default has occurred until all Secured Obligations have been satisfied in full and the Loan Documents have been terminated. Agent's foregoing appointment as Borrower's attorney in fact, and all of Agent's rights and powers, coupled with an interest, are irrevocable until all Secured Obligations have been fully repaid and performed and the Loan Documents have been terminated. Agent may, and at the direction of the Required

Lenders shall, exercise all rights and remedies with respect to the Collateral under the Loan Documents or otherwise available to it under the UCC and other applicable law, including the right to release, hold, sell, lease, liquidate, collect, realize upon, or otherwise dispose of all or any part of the Collateral and the right to occupy, utilize, process and commingle the Collateral. All Agent's rights and remedies shall be cumulative and not exclusive.

10.2 Collection; Foreclosure. Upon the occurrence and during the continuance of any Event of Default, Agent may, and at the direction of the Required Lenders shall, at any time or from time to time, apply, collect, liquidate, sell in one or more sales, lease or otherwise dispose of, any or all of the Collateral, in its then condition or following any commercially reasonable preparation or processing, in such order as Agent may elect. Any such sale may be made either at public or private sale at its place of business or elsewhere. Borrower agrees that any such public or private sale may occur upon ten (10)

calendar days' prior written notice to Borrower. Agent may require Borrower to assemble the Collateral and make it available to Agent at a place designated by Agent that is reasonably convenient to Agent and Borrower. The proceeds of any sale, disposition or other realization upon all or any part of the Collateral shall be applied by Agent in the following order of priorities:

- First, to Agent and the Lenders in an amount sufficient to pay in full Agent's and the Lenders' reasonable costs and professionals' and advisors' fees and expenses as described in Section 11.11;
- Second, to the Lenders in an amount equal to the then unpaid amount of the Secured Obligations (including principal, interest, and the Default Rate interest), in such order and priority as Agent may choose in its sole discretion; and
- Finally, after the full and final payment in Cash of all of the Secured Obligations (other than inchoate obligations), to any creditor holding a junior Lien on the Collateral, or to Borrower or its representatives or as a court of competent jurisdiction may direct.

Agent shall be deemed to have acted reasonably in the custody, preservation and disposition of any of the Collateral if it complies with the obligations of a secured party under the UCC.

10.3 No Waiver. Agent shall be under no obligation to marshal any of the Collateral for the benefit of Borrower or any other Person, and Borrower expressly waives all rights, if any, to require Agent to marshal any Collateral.

10.4 Cumulative Remedies. The rights, powers and remedies of Agent hereunder shall be in addition to all rights, powers and remedies given by statute or rule of law and are cumulative. The exercise of any one or more of the rights, powers and remedies provided herein shall not be construed as a waiver of or election of remedies with respect to any other rights, powers and remedies of Agent.

SECTION 11. MISCELLANEOUS

11.1 Severability. Whenever possible, each provision of this Agreement shall be interpreted in such manner as to be effective and valid under applicable law, but if any provision of this Agreement shall be prohibited by or invalid under such law, such provision shall be ineffective only to the extent and duration of such prohibition or invalidity, without invalidating the remainder of such provision or the remaining provisions of this Agreement.

11.2 Notice. Except as otherwise provided herein, any notice, demand, request, consent, approval, declaration, service of process or other communication (including the delivery of Financial Statements) that is required, contemplated, or permitted under the Loan Documents or with respect to the subject matter hereof shall be in writing, and shall be deemed to have been validly served, given, delivered, and received upon the earlier of: (i) the day of transmission by electronic mail or hand delivery or delivery by an overnight express service or overnight mail delivery service; or (ii) the third calendar day after deposit in the United States of America mails, with proper first class postage prepaid, in each case addressed to the party to be notified as follows:

(a) If to Agent:

HERCULES CAPITAL, INC.
Legal Department

Attention: Chief Legal Officer
400 Hamilton Avenue, Suite 310
Palo Alto, CA 94301
email:
Telephone:

(b) If to the Lenders:

HERCULES CAPITAL, INC.
Legal Department
Attention: Chief Legal Officer
400 Hamilton Avenue, Suite 310
Palo Alto, CA 94301
email:
Telephone:

(c) If to Borrower:

X4 PHARMACEUTICALS, INC.

Attention: Adam Mostafa and Brian Bowersox
61 North Beacon Street, 4th Floor
Boston, MA 02134
email:
Telephone:

Cooley LLP
55 Hudson Yards
New York, NY 10001
Email: ~~cmusa~~mtollini@cooley.com
Telephone: ~~212-479-6000~~ 202-962-8380

or to such other address as each party may designate for itself by like notice.

11.3 Entire Agreement; Amendments.

(a) This Agreement and the other Loan Documents constitute the entire agreement and understanding of the parties hereto in respect of the subject matter hereof and thereof, and supersede and replace in their entirety any prior proposals, term sheets, non-disclosure or confidentiality agreements, letters, negotiations or other documents or agreements, whether written or oral, with respect to the subject matter hereof or thereof (including Agent's revised proposal letter dated December 13, 2022 and the Non-Disclosure Agreement). For the avoidance of doubt, all security interests granted under the Original Agreement and the Original A&R LSA are hereby confirmed and ratified and shall continue to secure all Secured Obligations under this Agreement. None of the terms of this Agreement or any of the other Loan Documents may be amended except by an instrument executed by each of the parties hereto.

(b) Neither this Agreement, any other Loan Document, nor any terms hereof or thereof may be amended, supplemented or modified except in accordance with the provisions of this Section 11.3(b). The Required Lenders and Borrower party to the relevant Loan Document may, or, with the written consent of the Required Lenders, the Agent and the Borrower party to the relevant Loan Document may, from time to time, (i) enter into written amendments, supplements or modifications hereto and to the other Loan Documents for the purpose of adding any provisions to this Agreement or the other Loan Documents or changing in any manner the rights of the Lenders or of the Borrower

hereunder or thereunder or (ii) waive, on such terms and conditions as the Required Lenders or the Agent, as the case may be, may specify in such instrument, any of the requirements of this Agreement or the other Loan Documents or any ~~default~~Default or Event of Default and its consequences; provided, however, that no such waiver and no such amendment, supplement or modification shall (A) forgive the principal amount, extend the final scheduled date of maturity of any Loan, extend the scheduled date of any amortization payment in respect of any Term Loan Advance, or reduce the stated rate of any interest or fee payable hereunder, in each case without the written consent of each Lender directly affected thereby; (B) eliminate or reduce the voting rights of any Lender under this Section 11.3(b) without the written consent of such Lender; (C) reduce any percentage specified in the definition of Required Lenders, consent to the assignment or transfer by the Borrower of any of its rights and obligations under this Agreement and the other Loan Documents, release all or substantially all of the Collateral or release a Borrower from its obligations under the Loan Documents, in each case without the written consent of all Lenders; or (D) amend, modify or waive any provision of Section 11.17 without the written consent of the Agent. Any such waiver and any such amendment, supplement or modification shall apply equally to each Lender and shall be binding upon Borrower, the Lender, the Agent and all future holders of the Loans.

11.4 No Strict Construction. The parties hereto have participated jointly in the negotiation and drafting of this Agreement. In the event an ambiguity or question of intent or interpretation arises, this Agreement shall be construed as if drafted jointly by the parties hereto and no presumption or burden of proof shall arise favoring or disfavoring any party by virtue of the authorship of any provisions of this Agreement.

11.5 No Waiver. The powers conferred upon Agent and the Lenders by this Agreement are solely to protect its rights hereunder and under the other Loan Documents and its interest in the Collateral and shall not impose any duty upon Agent or the Lenders to exercise any such powers. No omission or delay by Agent or the Lenders at any time to enforce any right or remedy reserved to it, or to require performance of any of the terms, covenants or provisions hereof by Borrower at any time designated, shall be a waiver of any such right or remedy to which Agent or the Lenders is entitled, nor shall it in any way affect the right of Agent or the Lenders to enforce such provisions thereafter.

11.6 Survival. All agreements, representations and warranties contained in this Agreement and the other Loan Documents or in any document delivered pursuant hereto or thereto shall be for the benefit of Agent and the Lenders and shall survive the execution and delivery of this Agreement. Sections 6.3, 8.1 and 11.14 shall survive the termination of this Agreement.

11.7 Successors and Assigns. The provisions of this Agreement and the other Loan Documents shall inure to the benefit of and be binding on Borrower and its permitted assigns (if any). Borrower shall not assign its obligations under this Agreement or any of the other Loan Documents without Agent's express prior written consent, and any such attempted assignment shall be void and of no effect. Agent and the Lenders may assign, transfer, or endorse its rights hereunder and under the other Loan Documents without prior notice to Borrower, and all of such rights shall inure to the benefit of Agent's and the Lenders' successors and assigns; provided that as long as no Event of Default has occurred and is continuing, neither Agent nor any Lender may assign, transfer or endorse its rights hereunder or under the Loan Documents to any party that is a direct competitor of Borrower (as reasonably determined by Agent), it being acknowledged that in all cases, any transfer to an Affiliate of any Lender or Agent shall be allowed. Notwithstanding the foregoing, (x) in connection with any assignment by a Lender as a result of a forced divestiture at the request of any regulatory agency, the restrictions set forth herein shall not apply and Agent and the Lenders may assign, transfer or indorse its rights hereunder and under the other Loan Documents to any Person or party and (y) in connection with a Lender's own financing or securitization transactions, the restrictions set forth herein shall not apply and Agent and the Lenders may assign, transfer or indorse its rights hereunder and under the other Loan Documents to any Person or party providing such financing or formed to undertake such securitization transaction and any transferee of such Person or party upon the occurrence of a default, event of default or similar occurrence with respect to such financing or securitization transaction; provided that no such sale, transfer, pledge or assignment under this clause (y) shall release such Lender from any of its obligations hereunder or substitute any such Person or party for such Lender as a party hereto until Agent shall have received and accepted an effective assignment agreement from such Person or party in form satisfactory to Agent executed, delivered and fully completed by the applicable parties thereto, and shall have received such other information regarding such assignee as Agent reasonably shall require. The Agent, acting solely for this purpose as an agent of the Borrower, shall maintain at one of its offices in the United States a register for the recordation of the names and addresses of the Lender(s), and the Term Commitments of, and principal amounts (and stated interest) of the Loans owing to, each Lender pursuant to the terms hereof from time to time (the "Register"). The entries in the Register shall be conclusive absent manifest error, and the Borrower, the Agent and the Lender(s) shall treat each Person whose name is recorded in the Register pursuant to the terms hereof as a Lender hereunder for all purposes of this Agreement. The Register shall be available for inspection by the Borrower and any Lender, at any reasonable time and from time to time upon reasonable prior notice.

11.8 Governing Law. This Agreement and the other Loan Documents have been negotiated and delivered to Agent and the Lenders in the State of California, and shall have been accepted by Agent and the Lenders in the State of California. Payment to Agent and the Lenders by Borrower of the Secured Obligations is due in the State of California. This Agreement and the other Loan Documents shall be governed by, and construed and enforced in accordance with, the laws of the State of California, excluding conflict of laws principles that would cause the application of laws of any other jurisdiction.

11.9 Consent to Jurisdiction and Venue. All judicial proceedings (to the extent that the reference requirement of Section 11.10 is not applicable) arising in or under or related to this Agreement or any of the other

Loan Documents may be brought in any state or federal court located in the State of California. By execution and delivery of this Agreement, each party hereto generally and unconditionally: (a) consents to nonexclusive personal jurisdiction in Santa Clara County, State of California; (b) waives any objection as to jurisdiction or venue in Santa Clara County, State of California; (c) agrees not to assert any defense based on lack of jurisdiction or venue in the aforesaid courts; and (d) irrevocably agrees to be bound by any judgment rendered thereby in connection with this Agreement or the other Loan Documents. Service of process on any party hereto in any action arising out of or relating to this Agreement shall be effective if given in accordance with the requirements for notice set forth in Section 11.2, and shall be deemed effective and received as set forth in Section 11.2. Nothing herein shall affect the right to serve process in any other manner permitted by law or shall limit the right of either party to bring proceedings in the courts of any other jurisdiction.

11.10 Mutual Waiver of Jury Trial / Judicial Reference.

(a) Because disputes arising in connection with complex financial transactions are most quickly and economically resolved by an experienced and expert Person and the parties wish applicable state and federal laws to apply (rather than arbitration rules), the parties desire that their disputes be resolved by a judge applying such applicable laws. EACH OF BORROWER, AGENT AND THE LENDERS SPECIFICALLY WAIVES ANY RIGHT IT MAY HAVE TO TRIAL BY JURY OF ANY CAUSE OF ACTION, CLAIM, CROSS-CLAIM, COUNTERCLAIM, THIRD PARTY CLAIM OR ANY OTHER CLAIM (COLLECTIVELY, “CLAIMS”) ASSERTED BY BORROWER AGAINST AGENT, THE LENDERS OR THEIR RESPECTIVE ASSIGNEE OR BY AGENT, THE LENDERS OR THEIR RESPECTIVE ASSIGNEE AGAINST BORROWER. This waiver extends to all such Claims, including Claims that involve Persons other than Agent, Borrower and the Lenders; Claims that arise out of or are in any way connected to the relationship among Borrower, Agent and the Lenders; and any Claims for damages, breach of contract, tort, specific performance, or any equitable or legal relief of any kind, arising out of this Agreement, any other Loan Document.

(b) If the waiver of jury trial set forth in Section 11.10(a) is ineffective or unenforceable, the parties agree that all Claims shall be resolved by reference to a private judge sitting without a jury, pursuant to Code of Civil Procedure Section 638, before a mutually acceptable referee or, if the parties cannot agree, a referee selected by the Presiding Judge of the Santa Clara County, California. Such proceeding shall be conducted in Santa Clara County, California, with California rules of evidence and discovery applicable to such proceeding.

(c) In the event Claims are to be resolved by judicial reference, either party may seek from a court identified in Section 11.9, any prejudgment order, writ or other relief and have such prejudgment order, writ or other relief enforced to the fullest extent permitted by law notwithstanding that all Claims are otherwise subject to resolution by judicial reference.

11.11 Professional Fees. Borrower promises to pay Agent’s and the Lenders’ reasonable and documented fees and expenses necessary to finalize the loan documentation, including but not limited to reasonable and documented attorneys’ fees, UCC searches, filing costs, and other miscellaneous expenses. In addition, Borrower promises to pay any and all reasonable and documented attorneys’ and other professionals’ fees and expenses incurred by Agent and the Lenders after the Closing Date in connection with or related to: (a) the Loan; (b) the administration, collection, or enforcement of the Loan; (c) the amendment or modification of the Loan Documents; (d) any waiver, consent, release, or termination under the Loan Documents; (e) the protection, preservation, audit, field exam, sale, lease, liquidation, or disposition of Collateral or the exercise of remedies with respect to the Collateral; (f) any legal, litigation, administrative, arbitration, or out of court

proceeding in connection with or related to Borrower or the Collateral, and any appeal or review thereof; and (g) any bankruptcy, restructuring, reorganization, assignment for the benefit of creditors, workout, foreclosure, or other action related to Borrower, the Collateral, the Loan Documents, including representing Agent or the Lenders in any adversary proceeding or contested matter commenced or continued by or on behalf of Borrower's estate, and any appeal or review thereof.

11.12 Confidentiality. Agent and the Lenders acknowledge that certain items of Collateral and information provided to Agent and the Lenders, including items provided in connection with the Non-Disclosure Agreement, by Borrower are confidential and proprietary information of Borrower, if and to the extent such information either (x) is marked as confidential by Borrower at the time of disclosure, or (y) should reasonably be understood to be confidential (the "Confidential Information"). Accordingly, Agent and the Lenders agree that any Confidential Information it may obtain during the term of this Agreement shall not be disclosed to any other Person or entity in any manner whatsoever,

in whole or in part, without the prior written consent of Borrower, except that Agent and Lenders may disclose any such information: (a) to its own directors, officers, employees, accountants, counsel and other professional advisors and to its Affiliates if Agent or Lenders in their sole discretion determines that any such party should have access to such information in connection with such party's responsibilities in connection with the Loan or this Agreement and, provided that such recipient of such Confidential Information either (i) agrees to be bound by the confidentiality provisions of this paragraph or (ii) is otherwise subject to confidentiality restrictions that reasonably protect against the disclosure of Confidential Information; (b) if such information is generally available to the public through no fault of Agent or Lenders; (c) if required or appropriate in any report, statement or testimony submitted to any governmental authority having or claiming to have jurisdiction over Agent or Lenders; (d) if required or appropriate in response to any summons or subpoena or in connection with any litigation, to the extent permitted or deemed advisable by Agent's or Lenders' counsel; (e) to comply with any legal requirement or law applicable to Agent or Lenders; (f) to the extent reasonably necessary in connection with the exercise of any right or remedy under any Loan Document, including Agent's sale, lease, or other disposition of Collateral after an Event of Default; (g) to any participant or assignee of Agent or Lenders or any prospective participant or assignee; provided, that such participant or assignee or prospective participant or assignee agrees in writing to be bound by this Section 11.12 prior to disclosure; or (h) otherwise with the prior consent of Borrower; provided, that any disclosure made in violation of this Agreement shall not affect the obligations of Borrower or any of its Affiliates or any guarantor under this Agreement or the other Loan Documents. Agent's and Lenders' obligations under this Section 11.12 shall supersede all of their respective obligations under the Non-Disclosure Agreement.

11.13 Assignment of Rights. Borrower acknowledges and understands that Agent or the Lenders may, subject to Section 11.7, sell and assign all or part of its interest hereunder and under the Loan Documents to any Person or entity (an "Assignee"). After such assignment the term "Agent" or "Lender" as used in the Loan Documents shall mean and include such Assignee, and such Assignee shall be vested with all rights, powers and remedies of Agent and the Lenders hereunder with respect to the interest so assigned; but with respect to any such interest not so transferred, Agent and the Lenders shall retain all rights, powers and remedies hereby given. No such assignment by Agent or the Lenders shall relieve Borrower of any of its obligations hereunder. The Lenders agree that in the event of any transfer by it of the Note(s)(if any), it will endorse thereon a notation as to the portion of the principal of the Note(s), which shall have been paid at the time of such transfer and as to the date to which interest shall have been last paid thereon.

11.14 Revival of Secured Obligations. This Agreement and the Loan Documents shall remain in full force and effect and continue to be effective if any petition is filed by or against Borrower for liquidation or reorganization, if Borrower becomes insolvent or makes an assignment for the benefit of creditors, if a receiver or trustee is appointed for all or any significant part of Borrower's assets, or if any payment or transfer of Collateral is recovered from Agent or the Lenders. The Loan Documents and the Secured Obligations and Collateral security shall continue to be effective, or shall be revived or reinstated, as the case may be, if at any time payment and performance of the Secured Obligations or any transfer of Collateral to Agent, or any part thereof is rescinded, avoided or avoidable, reduced in amount, or must otherwise be restored or returned by, or is recovered from, Agent, the Lenders or by any obligee of the Secured Obligations, whether as a "voidable preference," "fraudulent conveyance," or otherwise, all as though such payment, performance, or transfer of Collateral had not been made. In the event that any payment, or any part thereof, is rescinded, reduced, avoided, avoidable, restored, returned, or recovered, the Loan Documents and the Secured Obligations shall be deemed, without any further action or documentation, to have been revived and reinstated except to the extent of the full, final, and indefeasible payment to Agent or the Lenders in Cash.

11.15 Counterparts. This Agreement and any amendments, waivers, consents or supplements hereto may be executed in any number of counterparts, and by different parties hereto in separate counterparts, each of which when so delivered shall be deemed an original, but all of which counterparts shall constitute but one and the same instrument.

11.16 No Third Party Beneficiaries. No provisions of the Loan Documents are intended, nor will be interpreted, to provide or create any third-party beneficiary rights or any other rights of any kind in any Person other than Agent, the Lenders and Borrower unless specifically provided otherwise herein, and, except as otherwise so provided, all provisions of the Loan Documents will be personal and solely among Agent, the Lenders and the Borrower.

11.17 Agency.

(a) Lender hereby irrevocably appoints Hercules Capital, Inc. to act on its behalf as the Agent hereunder and under the other Loan Documents and authorizes the Agent to take such actions on its behalf and to exercise such powers as are delegated to the Agent by the terms hereof or thereof, together with such actions and powers as are reasonably incidental thereto.

(b) Lender agrees to indemnify the Agent in its capacity as such (to the extent not reimbursed by Borrower and without limiting the obligation of Borrower to do so), according to its respective Term Commitment percentages (based upon the total outstanding Term Loan Commitments) in effect on the date on which indemnification is sought under this Section 11.17, from and against any and all liabilities, obligations, losses, damages, penalties, actions, judgments, suits, costs, expenses or disbursements of any kind whatsoever that may at any time be imposed on, incurred by or asserted against the Agent in any way relating to or arising out of, this Agreement, any of the other Loan Documents or any documents contemplated by or referred to herein or therein or the transactions contemplated hereby or thereby or any action taken or omitted by the Agent under or in connection with any of the foregoing; The agreements in this Section shall survive the payment of the Loans and all other amounts payable hereunder.

(c) Agent in Its Individual Capacity. The Person serving as the Agent hereunder shall have the same rights and powers in its capacity as a Lender as any other Lender and may exercise the same as though it were

not the Agent and the term “Lender” shall, unless otherwise expressly indicated or unless the context otherwise requires, include each such Person serving as Agent hereunder in its individual capacity.

(d) Exculpatory Provisions. The Agent shall have no duties or obligations except those expressly set forth herein and in the other Loan Documents. Without limiting the generality of the foregoing, the Agent shall not:

(i) be subject to any fiduciary or other implied duties, regardless of whether any default or any Event of Default has occurred and is continuing;

(ii) have any duty to take any discretionary action or exercise any discretionary powers, except discretionary rights and powers expressly contemplated hereby or by the other Loan Documents that the Agent is required to exercise as directed in writing by the Lenders, provided that the Agent shall not be required to take any action that, in its opinion or the opinion of its counsel, may expose the Agent to liability or that is contrary to any Loan Document or applicable law; and

(iii) except as expressly set forth herein and in the other Loan Documents, have any duty to disclose, and the Agent shall not be liable for the failure to disclose, any information relating to the Borrower or any of its Affiliates that is communicated to or obtained by any Person serving as the Agent or any of its Affiliates in any capacity.

(e) The Agent shall not be liable for any action taken or not taken by it (i) with the consent or at the request of the Lenders or as the Agent shall believe in good faith shall be necessary, under the circumstances or (ii) in the absence of its own gross negligence or willful misconduct.

(f) The Agent shall not be responsible for or have any duty to ascertain or inquire into (i) any statement, warranty or representation made in or in connection with this Agreement or any other Loan Document, (ii) the contents of any certificate, report or other document delivered hereunder or thereunder or in connection herewith or therewith, (iii) the performance or observance of any of the covenants, agreements or other terms or conditions set forth herein or therein or the occurrence of any default or Event of Default, (iv) the validity, enforceability, effectiveness or genuineness of this Agreement, any other Loan Document or any other agreement, instrument or document or (v) the satisfaction of any condition set forth in Section 4 or elsewhere herein, other than to confirm receipt of items expressly required to be delivered to the Agent.

(g) Reliance by Agent. Agent may rely, and shall be fully protected in acting, or refraining to act, upon, any resolution, statement, certificate, instrument, opinion, report, notice, request, consent, order, bond or other paper or document that it has no reason to believe to be other than genuine and to have been signed or presented by the proper party or parties or, in the case of cables, telecopies and telexes, to have been sent by the proper party or parties. In the absence of its gross negligence or willful misconduct, Agent may conclusively rely, as to the truth of the statements and the correctness of the opinions expressed therein, upon any certificates or opinions furnished to Agent and conforming to the requirements of the Agreement or any of the other Loan Documents. Agent may consult with counsel, and any opinion or legal advice of such counsel shall be full and complete authorization and protection in respect of any action taken, not taken or suffered by Agent hereunder or under any Loan Documents in accordance therewith. Agent shall have the right at any time to seek instructions concerning the administration of the Collateral from any court of competent jurisdiction. Agent shall not be under any obligation to exercise any of the rights or powers granted to Agent by this Agreement, the

Agreement and the other Loan Documents at the request or direction of Lenders unless Agent shall have been provided by each Lender with adequate security and indemnity against the costs, expenses and liabilities that may be incurred by it in compliance with such request or direction.

11.18 Publicity. None of the parties hereto nor any of its respective member businesses and Affiliates shall, without the other parties' prior written consent (which shall not be unreasonably withheld or delayed), publicize or use (a) the other party's name (including a brief description of the relationship among the parties hereto), logo or hyperlink to such other parties' web site, separately or together, in written and oral presentations, advertising, promotional and marketing materials, client lists, public relations materials or on its web site (together, the "Publicity Materials"); (b) the names of officers of such other parties in the Publicity Materials; and (c) such other parties' name,

trademarks, servicemarks in any news or press release concerning such party; provided however, notwithstanding anything to the contrary herein, no such consent shall be required (i) to the extent necessary to comply with the requests of any regulators, legal requirements or laws applicable to such party, pursuant to any listing agreement with any national securities exchange (so long as such party provides prior notice to the other party hereto to the extent reasonably practicable) and (ii) to comply with Section 11.12.

11.19 Multiple Borrowers.

(a) Borrower's Agent. Each of the Borrowers hereby irrevocably appoints the Company as its agent, attorney-in-fact and legal representative for all purposes, including requesting disbursement of the Term Loan Advances and receiving account statements and other notices and communications to Borrowers (or any of them) from the Agent or any Lender. The Agent may rely, and shall be fully protected in relying, on any request for the Term Loan Advances, disbursement instruction, report, information or any other notice or communication made or given by the Company, whether in its own name or on behalf of one or more of the other Borrowers, and the Agent shall not have any obligation to make any inquiry or request any confirmation from or on behalf of any other Borrower as to the binding effect on it of any such request, instruction, report, information, other notice or communication, nor shall the joint and several character of the Borrowers' obligations hereunder be affected thereby.

(b) Waivers. Each Borrower hereby waives: (i) any right to require the Agent to institute suit against, or to exhaust its rights and remedies against, any other Borrower or any other person, or to proceed against any property of any kind which secures all or any part of the Secured Obligations, or to exercise any right of offset or other right with respect to any reserves, credits or deposit accounts held by or maintained with the Agent or any Indebtedness of the Agent or any Lender to any other Borrower, or to exercise any other right or power, or pursue any other remedy the Agent or any Lender may have; (ii) any defense arising by reason of any disability or other defense of any other Borrower or any guarantor or any endorser, co-maker or other person, or by reason of the cessation from any cause whatsoever of any liability of any other Borrower or any guarantor or any endorser, co-maker or other person, with respect to all or any part of the Secured Obligations, or by reason of any act or omission of the Agent or others which directly or indirectly results in the discharge or release of any other Borrower or any guarantor or any other person or any Secured Obligations or any security therefor, whether by operation of law or otherwise; (iii) any defense arising by reason of any failure of the Agent to obtain, perfect, maintain or keep in force any Lien on, any property of any Borrower or any other person; (iv) any defense based upon or arising out of any bankruptcy, insolvency, reorganization, arrangement, readjustment of

debt, liquidation or dissolution proceeding commenced by or against any other Borrower or any guarantor or any endorser, co-maker or other person, including without limitation any discharge of, or bar against collecting, any of the Secured Obligations (including without limitation any interest thereon), in or as a result of any such proceeding. Until all of the Secured Obligations have been paid, performed, and discharged in full, nothing shall discharge or satisfy the liability of any Borrower hereunder except the full performance and payment of all of the Secured Obligations. If any claim is ever made upon the Agent for repayment or recovery of any amount or amounts received by the Agent in payment of or on account of any of the Secured Obligations, because of any claim that any such payment constituted a preferential transfer or fraudulent conveyance, or for any other reason whatsoever, and the Agent repays all or part of said amount by reason of any judgment, decree or order of any court or administrative body having jurisdiction over the Agent or any of its property, or by reason of any settlement or compromise of any such claim effected by the Agent with any such claimant (including without limitation the any other Borrower), then and in any such event, each Borrower agrees that any such judgment, decree, order, settlement and compromise shall be binding upon such Borrower, notwithstanding any revocation or release of this Agreement or the cancellation of any note or other instrument evidencing any of the Secured Obligations, or any release of any of the Secured Obligations, and each Borrower shall be and remain liable to the Agent and the Lenders under this Agreement for the amount so repaid or recovered, to the same extent as if such amount had never originally been received by the Agent or any Lender, and the provisions of this sentence shall survive, and continue in effect, notwithstanding any revocation or release of this Agreement. Each Borrower hereby expressly and unconditionally waives all rights of subrogation, reimbursement and indemnity of every kind against any other Borrower, and all rights of recourse to any assets or property of any other Borrower, and all rights to any collateral or security held for the payment and performance of any Secured Obligations, including (but not limited to) any of the foregoing rights which Borrower may have under any present or future document or agreement with any other Borrower or other person, and including (but not limited to) any of the foregoing rights which any Borrower may have under any equitable doctrine of subrogation, implied contract, or unjust enrichment, or any other equitable or legal doctrine.

(c) Consents. Each Borrower hereby consents and agrees that, without notice to or by Borrower and without affecting or impairing in any way the obligations or liability of Borrower hereunder, the Agent may, from time to time before or after revocation of this Agreement, do any one or more of the following in its sole and absolute discretion: (i) accept partial payments of, compromise or settle, renew, extend the time for the payment, discharge, or performance of, refuse to enforce, and release all or any parties to, any or all of the Secured Obligations; (ii) grant any other indulgence to any Borrower or any other Person in respect of any or all of the Secured Obligations or any other matter; (iii) accept, release, waive, surrender, enforce, exchange, modify, impair, or extend the time for the performance, discharge, or payment of, any and all property of any kind securing any or all of the Secured Obligations or any guaranty of any or all of the Secured Obligations, or on which the Agent at any time may have a Lien, or refuse to enforce its rights or make any compromise or settlement or agreement therefor in respect of any or all of such property; (iv) substitute or add, or take any action or omit to take any action which results in the release of, any one or more other Borrowers or any endorsers or guarantors of all or any part of the Secured Obligations, including, without limitation one or more parties to this Agreement, regardless of any destruction or impairment of any right of contribution or other right of Borrower; (v) apply any sums received from any other Borrower, any guarantor, endorser, or co-signer, or from the disposition of any Collateral or security, to any Indebtedness whatsoever owing from such person or secured by such Collateral or security, in such manner and order as the Agent determines in its sole discretion, and regardless of whether such Indebtedness is part of the Secured Obligations, is secured, or is due and payable. Each Borrower consents and agrees that the Agent shall be under no obligation to marshal any assets in favor of

Borrower, or against or in payment of any or all of the Secured Obligations. Each Borrower further consents and agrees that the Agent shall have no duties or responsibilities whatsoever with respect to any property securing any or all of the Secured Obligations. Without limiting the generality of the foregoing, the Agent shall have no obligation to monitor, verify, audit, examine, or obtain or maintain any insurance with respect to, any property securing any or all of the Secured Obligations.

(d) Independent Liability. Each Borrower hereby agrees that one or more successive or concurrent actions may be brought hereon against such Borrower, in the same action in which any other Borrower may be sued or in separate actions, as often as deemed advisable by Agent. Each Borrower is fully aware of the financial condition of each other Borrower and is executing and delivering this Agreement based solely upon its own independent investigation of all matters pertinent hereto, and such Borrower is not relying in any manner upon any representation or statement of the Agent or any Lender with respect thereto. Each Borrower represents and warrants that it is in a position to obtain, and each Borrower hereby assumes full responsibility for obtaining, any additional information concerning any other Borrower's financial condition and any other matter pertinent hereto as such Borrower may desire, and such Borrower is not relying upon or expecting the Agent to furnish to it any information now or hereafter in the Agent's possession concerning the same or any other matter.

(e) Subordination. All Indebtedness of a Borrower now or hereafter arising held by another Borrower is subordinated to the Secured Obligations and the Borrower holding the Indebtedness shall take all actions reasonably requested by Agent to effect, to enforce and to give notice of such subordination.

11.20 Amendment and Restatement. This Agreement amends and restates in its entirety the Original A&R LSA effective as of the date hereof. Anything contained herein to the contrary notwithstanding, this Agreement is not intended to and shall not serve to effect a novation of the "Secured Obligations" (as defined in the Original A&R LSA). Instead, it is the express intention of the parties hereto to reaffirm the indebtedness, obligations and liabilities created under the Original A&R LSA which is secured by the Collateral pursuant to the terms of the applicable Loan Documents. Borrower ratifies, affirms and confirms that the liens and security interests granted pursuant to the applicable Loan Documents, including without limitation the Pledge Agreements, secure the applicable indebtedness, liabilities and obligations of Borrower to Agent and the Lenders under the Original A&R LSA, as amended and restated by this Agreement, the Loan Documents shall continue in full force and effect in accordance with their terms unless otherwise amended by the parties thereto, and that the term "Secured Obligations" as used in the Loan Documents (or any other term used therein to describe or refer to the indebtedness, liabilities and obligations of Borrower to Agent and the Lenders) includes, without limitation, the indebtedness, liabilities and obligations of Borrower under this Agreement, and under the Original A&R LSA, as amended and restated hereby, as the same further may be amended, modified, supplemented and/or restated from time to time and Borrower assumes all such Secured Obligations. Pursuant to the definition of Borrower in this Agreement, the security interests granted pursuant to Section 3.1 and by the Pledge Agreements are granted by the Company and each other Person constituting the Borrower in the Company's and each such Person's respective right, title and interest in and to any and all presently existing and hereafter created or acquired Collateral. The Loan Documents and all agreements, instruments and documents executed or delivered in connection with any of the foregoing shall each be deemed to be amended to the extent necessary to give effect to the provisions of this Agreement. Each reference to the "Loan and Security Agreement" in any Loan Document shall mean and be a reference to this Agreement (as further amended, restated, supplemented or otherwise modified from time to time). Cross-references in the Loan Documents to particular section numbers in the Original

A&R LSA shall be deemed to be cross-references to the corresponding sections, as applicable, of this Agreement.

11.21 Participations. Each Lender that sells a participation shall, acting solely for this purpose as a non-fiduciary agent of the Borrower, maintain a register on which it enters the name and address of each participant and the principal amounts (and stated interest) of each participant's interest in the Loans or other obligations under the Loan Documents (the "Participant Register"); provided that no Lender shall have any obligation to disclose all or any portion of the Participant Register (including the identity of any participant or any information relating to a participant's interest in any commitments,

loans, its other obligations under any Loan Document) to any Person except to the extent that such disclosure is necessary to establish that such commitment, loan, letter of credit or other obligation is in registered form under Section 5f.103-1(c) of the United States Treasury Regulations. The entries in the Participant Register shall be conclusive absent manifest error, and such Lender shall treat each Person whose name is recorded in the Participant Register as the owner of such participation for all purposes of this Agreement notwithstanding any notice to the contrary. For the avoidance of doubt, the Agent (in its capacity as Agent) shall have no responsibility for maintaining a Participant Register.

11.22 Electronic Execution of Certain Other Documents. The words "execution," "execute", "signed," "signature," and words of like import in or related to any document to be signed in connection with this Agreement and the transactions contemplated hereby (including without limitation assignments, assumptions, amendments, waivers and consents) shall be deemed to include electronic signatures, the electronic matching of assignment terms and contract formations on electronic platforms approved by the Agent, or the keeping of records in electronic form, each of which shall be of the same legal effect, validity or enforceability as a manually executed signature or the use of a paper-based recordkeeping system, as the case may be, to the extent and as provided for in any applicable law, including the Federal Electronic Signatures in Global and National Commerce Act, the California Uniform Electronic Transaction Act, or any other similar state laws based on the Uniform Electronic Transactions Act.

(SIGNATURES TO FOLLOW)

IN WITNESS WHEREOF, Borrower, Agent and Lender have duly executed and delivered this Second Amended and Restated Loan and Security Agreement as of the day and year first above written.

Table of Exhibits and Schedules
Addendum 1: Taxes; Increased Costs

ADDENDUM 1 to

SECOND AMENDED AND RESTATED LOAN AND SECURITY AGREEMENT

TAXES; INCREASED COSTS

1. **Defined Terms**. For purposes of this Addendum 1:

a. "**Connection Income Taxes**" means Other Connection Taxes that are imposed on or measured by net income (however denominated) or that are franchise Taxes or branch profits Taxes.

b. "**Excluded Taxes**" means any of the following Taxes imposed on or with respect to a Recipient or required to be withheld or deducted from a payment to a Recipient, (i) Taxes imposed on or measured by net income (however denominated), franchise Taxes, and branch profits Taxes, in each case, (A) imposed as a result of such Recipient being organized under the laws of, or having its principal office or, in the case of any Lender, its applicable lending office located in, the jurisdiction imposing such Tax (or any political subdivision thereof) or (B) that are Other Connection Taxes, (ii) in the case of a Lender, U.S. federal withholding Taxes imposed on amounts payable to or for the account of such Lender with respect to an applicable interest in a Loan or Term Commitment pursuant to a law in effect on the date on which (A) such Lender acquires such interest in the Loan or Term Commitment or (B) such Lender changes its lending office, except in each case to the extent that, pursuant to Section 2 or Section 4 of this Addendum 1, amounts with respect to such Taxes were payable either to such Lender's assignor immediately before such Lender became a party hereto or to such Lender immediately before it changed its lending office, (iii) Taxes attributable to such Recipient's failure to comply with Section 7 of this Addendum 1 and (iv) any withholding Taxes imposed under FATCA.

c. "**FATCA**" means Sections 1471 through 1474 of the Code, as of the date of this Agreement (or any amended or successor version that is substantively comparable and not materially more onerous to comply with), any current or future regulations or official interpretations thereof, any agreements entered into pursuant to Section 1471(b)(1) of the Code, and any fiscal or regulatory legislation, rules or practices adopted pursuant to any intergovernmental agreement, treaty or convention among governmental authorities and implementing such Sections of the Code.

d. "**Foreign Lender**" means a Lender that is not a U.S. Person.

e. "**Indemnified Taxes**" means (i) Taxes, other than Excluded Taxes, imposed on or with respect to any payment made by or on account of any obligation of the Borrower under any Loan Document and (ii) to the extent not otherwise described in clause (i), Other Taxes.

f. "**Other Connection Taxes**" means, with respect to any Recipient, Taxes imposed as a result of a present or former connection between such Recipient and the jurisdiction imposing such Tax (other than connections arising from such Recipient having executed, delivered, become a party to, performed its obligations under, received payments under, received or perfected a security interest under, engaged in any other transaction pursuant to or enforced any Loan Document, or sold or assigned an interest in any Loan or Loan Document).

g. "**Other Taxes**" means all present or future stamp, court or documentary, intangible, recording, filing or similar Taxes that arise from any payment made under, from the execution, delivery, performance, enforcement or

registration of, from the receipt or perfection of a security interest under, or otherwise with respect to, any Loan Document, except any such Taxes that are Other Connection Taxes imposed with respect to an assignment.

h. "**Recipient**" means the Agent or any Lender, as applicable.

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i. "**Taxes**" means all present or future taxes, levies, imposts, duties, deductions, withholdings (including backup withholding), assessments, fees or other charges imposed by any governmental authority, including any interest, additions to tax or penalties applicable thereto.

j. "**Withholding Agent**" means the Borrower and the Agent.

2. **Payments Free of Taxes**. Any and all payments by or on account of any obligation of the Borrower under any Loan Document shall be made without deduction or withholding for any Taxes, except as required by applicable law. If any applicable law (as determined in the good faith discretion of an applicable Withholding Agent) requires the deduction or withholding of any Tax from any such payment by a Withholding Agent, then the applicable Withholding Agent shall be entitled to make such deduction or withholding and shall timely pay the full amount deducted or withheld to the relevant governmental authority in accordance with applicable law and, if such Tax is an Indemnified Tax, then the sum payable by the Borrower shall be increased as necessary so that after such deduction or withholding has been made (including such deductions and withholdings applicable to additional sums payable under this Section 2 or Section 4 of this Addendum 1) the applicable Recipient receives an amount equal to the sum it would have received had no such deduction or withholding been made.

3. **Payment of Other Taxes by Borrower**. The Borrower shall timely pay to the relevant governmental authority in accordance with applicable law, or at the option of the Agent timely reimburse it for the payment of, any Other Taxes.

4. **Indemnification by Borrower**. The Borrower shall indemnify each Recipient, within 10 days after demand therefor, for the full amount of any Indemnified Taxes (including Indemnified Taxes imposed or asserted on or attributable to amounts payable under Section 2 of this Addendum 1 or this Section 4) payable or paid by such Recipient or required to be withheld or deducted from a payment to such Recipient and any reasonable expenses arising therefrom or with respect thereto, whether or not such Indemnified Taxes were correctly or legally imposed or asserted by the relevant governmental authority. A certificate as to the amount of such payment or liability delivered to the Borrower by a Lender (with a copy to the Agent), or by the Agent on its own behalf or on behalf of a Lender, shall be conclusive absent manifest error. In addition, the Borrower agrees to pay, and to save the Agent and any Lender harmless from, any and all liabilities with respect to, or resulting from any delay in paying, any and all excise, sales or other similar taxes (excluding taxes

imposed on or measured by the net income of the Agent or such Lender) that may be payable or determined to be payable with respect to any of the Collateral or this Agreement.

5. **Indemnification by the Lenders**. Each Lender shall severally indemnify the Agent, within 10 days after demand therefor, for (a) any Indemnified Taxes attributable to such Lender (but only to the extent that the Borrower has not already indemnified the Agent for such Indemnified Taxes and without limiting the obligation of the Borrower to do so), (b) any Taxes attributable to such Lender's failure to comply with the provisions

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of Section 11.21 of the Agreement relating to the maintenance of a Participant Register and (c) any Excluded Taxes attributable to such Lender, in each case, that are payable or paid by the Agent in connection with any Loan Document, and any reasonable expenses arising therefrom or with respect thereto, whether or not such Taxes were correctly or legally imposed or asserted by the relevant governmental authority. A certificate as to the amount of such payment or liability delivered to any Lender by the Agent shall be conclusive absent manifest error. Each Lender hereby authorizes the Agent to set off and apply any and all amounts at any time owing to such Lender under any Loan Document or otherwise payable by the Agent to the Lender from any other source against any amount due to the Agent under this Section 5.

6. **Evidence of Payments**. As soon as practicable after any payment of Taxes by the Borrower to a governmental authority pursuant to the provisions of this Addendum 1, the Borrower shall deliver to the Agent the original or a certified copy of a receipt issued by such governmental authority evidencing such payment, a copy of the return reporting such payment or other evidence of such payment reasonably satisfactory to the Agent.

7. **Status of Lenders**.

a. Any Lender that is entitled to an exemption from or reduction of withholding Tax with respect to payments made under any Loan Document shall deliver to the Borrower and the Agent, at the time or times reasonably requested by the Borrower or the Agent, such properly completed and executed documentation reasonably requested by the Borrower or the Agent as will permit such payments to be made without withholding or at a reduced rate of withholding. In addition, any Lender, if reasonably requested by the Borrower or the Agent, shall deliver such other documentation prescribed by applicable law or reasonably requested by the Borrower or the Agent as will enable the Borrower or the Agent to determine whether or not such Lender is subject to backup withholding or information reporting requirements. Notwithstanding anything to the contrary in the preceding two sentences, the completion, execution and submission of such documentation (other than such documentation set forth in Sections 7(b)(i), 7(b)(ii) and 7(b)(iv) of this Addendum 1) shall not be required if in the Lender's reasonable judgment such completion, execution or submission would subject such Lender to any material unreimbursed cost or expense or would materially prejudice the legal or commercial position of such Lender.

b. Without limiting the generality of the foregoing, in the event that the Borrower is a U.S. Person,

i. any Lender that is a U.S. Person shall deliver to the Borrower and the Agent on or prior to the date on which such Lender becomes a Lender under this Agreement (and from time to time thereafter upon the reasonable request of the Borrower or the Agent), executed copies of IRS Form W-9 certifying that such Lender is exempt from U.S. federal backup withholding tax;

ii. any Foreign Lender shall, to the extent it is legally entitled to do so, deliver to the Borrower and the Agent (in such number of copies as shall be requested by the recipient) on or prior to the date on which such Foreign Lender becomes a Lender under this Agreement (and from time to time thereafter upon the reasonable request of the Borrower or the Agent), whichever of the following is applicable:

A. in the case of a Foreign Lender claiming the benefits of an income tax treaty to which the United States is a party (x) with respect to payments of interest under any Loan Document, executed copies of IRS Form W-8BEN or IRS Form W-8BEN-E establishing an exemption from, or reduction of, U.S. federal withholding Tax pursuant to the "interest" article of such tax treaty and (y) with

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respect to any other applicable payments under any Loan Document, IRS Form W-8BEN or IRS Form W-8BEN-E establishing an exemption from, or reduction of, U.S. federal withholding Tax pursuant to the "business profits" or "other income" article of such tax treaty;

B. executed copies of IRS Form W-8ECI;

C. in the case of a Foreign Lender claiming the benefits of the exemption for portfolio interest under Section 881(c) of the Code, (x) a certificate substantially in the form of Exhibit I-1 to the effect that such Foreign Lender is not a "bank" within the meaning of Section 881(c)(3)(A) of the Code, a "10 percent shareholder" of the Borrower within the meaning of Section 871(h)(3)(B) of the Code, or a "controlled foreign corporation" related to the Borrower as described in Section 881(c)(3)(C) of the Code (a "**U.S. Tax Compliance Certificate**") and (y) executed copies of IRS Form W-8BEN or IRS Form W-8BEN-E; or

D. to the extent a Foreign Lender is not the beneficial owner, executed copies of IRS Form W-8IMY, accompanied by IRS Form W-8ECI, IRS Form W-8BEN, IRS Form W-8BEN-E, a U.S. Tax Compliance Certificate substantially in the form of Exhibit I-2 or Exhibit I-3, IRS Form W-9, and/or other certification documents from each beneficial owner, as applicable; provided that if the Foreign Lender is a partnership and one or more direct or indirect partners of such Foreign Lender are claiming the portfolio interest exemption, such Foreign Lender may provide a U.S. Tax Compliance Certificate substantially in the form of Exhibit I-4 on behalf of each such direct and indirect partner;

iii. any Foreign Lender shall, to the extent it is legally entitled to do so, deliver to the Borrower and the Agent (in such number of copies as shall be requested by the recipient) on or prior to the date on which such Foreign Lender becomes a Lender under this Agreement (and from time to time thereafter upon the reasonable request of the Borrower or the Agent), executed copies of any other form prescribed by applicable law as a basis for claiming exemption from or a reduction in U.S. federal withholding Tax, duly completed, together with such

supplementary documentation as may be prescribed by applicable law to permit the Borrower or the Agent to determine the withholding or deduction required to be made; and iv. if a payment made to a Lender under any Loan Document would be subject to U.S. federal withholding Tax imposed by FATCA if such Lender were to fail to comply with the applicable reporting requirements of FATCA (including those contained in Section 1471(b) or 1472(b) of the Code, as applicable), such Lender shall deliver to the Borrower and the Agent at the time or times prescribed by law and at such time or times reasonably requested by the Borrower or the Agent such documentation prescribed by applicable law (including as prescribed by Section 1471(b)(3)(C)(i) of the Code) and such additional documentation reasonably requested by the Borrower or the Agent as may be necessary for the Borrower and the Agent to comply with their obligations under FATCA and to determine that such Lender has complied with such Lender's obligations under FATCA or to determine the amount, if any, to deduct and withhold from such payment. Solely for purposes of this clause (iv), "FATCA" shall include any amendments made to FATCA after the date of this Agreement.

c. Each Lender agrees that if any form or certification it previously delivered expires or becomes obsolete or inaccurate in any respect, it shall update such form or certification or promptly notify the Borrower and the Agent in writing of its legal inability to do so.

8. **Treatment of Certain Refunds**. If any party determines, in its sole discretion exercised in good faith, that it has received a refund of any Taxes as to which it has been indemnified pursuant to the provisions of this Addendum 1 (including by the payment of additional amounts pursuant to the provisions of this Addendum

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1), it shall pay to the indemnifying party an amount equal to such refund (but only to the extent of indemnity payments made under the provisions of this Addendum 1 with respect to the Taxes giving rise to such refund), net of all out-of-pocket expenses (including Taxes) of such indemnified party and without interest (other than any interest paid by the relevant governmental authority with respect to such refund). Such indemnifying party, upon the request of such indemnified party, shall repay to such indemnified party the amount paid over pursuant to this Section 8 (plus any penalties, interest or other charges imposed by the relevant governmental authority) in the event that such indemnified party is required to repay such refund to such governmental authority. Notwithstanding anything to the contrary in this Section 8, in no event will the indemnified party be required to pay any amount to an indemnifying party pursuant to this Section 8 the payment of which would place the indemnified party in a less favorable net after-Tax position than the indemnified party would have been in if the Tax subject to indemnification and giving rise to such refund had not been deducted, withheld or otherwise imposed and the indemnification payments or additional amounts with respect to such Tax had never been paid. This Section 8 shall not be construed to require any indemnified party to make available its Tax returns (or any other

information relating to its Taxes that it deems confidential) to the indemnifying party or any other Person.

9. **Increased Costs**. If any change in applicable law shall subject any Recipient to any Taxes (other than (A) Indemnified Taxes, (B) Taxes described in clauses (ii) through (iv) of the definition of Excluded Taxes and (C) Connection Income Taxes) on its loans, loan principal, commitments, or other obligations, or its deposits, reserves, other liabilities or capital attributable thereto, and the result shall be to increase the cost to such Recipient of making, converting to, continuing or maintaining any Term Loan or of maintaining its obligation to make any such Loan, or to reduce the amount of any sum received or receivable by such Recipient (whether of principal, interest or any other amount), then, upon the request of such Recipient, the Borrower will pay to such Recipient such additional amount or amounts as will compensate such Recipient for such additional costs incurred or reduction suffered.

10. **Survival**. Each party's obligations under the provisions of this Addendum 1 shall survive the resignation or replacement of the Agent or any assignment of rights by, or the replacement of, a Lender, the termination of the Term Commitments and the repayment, satisfaction or discharge of all obligations under any Loan Document.

EXHIBIT A
ADVANCE REQUEST

To: Agent: Date: _____

Hercules Capital, Inc. (the "Agent")
400 Hamilton Avenue, Suite 310
Palo Alto, CA 94301
email:
Attn:

X4 Pharmaceuticals, Inc., a Delaware corporation ("Borrower") hereby requests ~~from Hercules Capital, Inc. ("Lender")~~ ~~an~~Agent to cause Lenders to make a [[Tranche 2 Advance][Tranche 3 Advance][Tranche 4 Advance][Tranche 5 Advance][Tranche 6 Advance]] in the amount of _____ Dollars ($_____) on _____, _____ (the "Advance Date") pursuant to the Second Amended and Restated Loan and Security Agreement among Borrower, Agent and Lender (the "Agreement"). Capitalized words and other terms used but not otherwise defined herein are used with the same meanings as defined in the Agreement.

Please:

(a) Issue a check payable to Borrower _____

or

(b) Wire Funds to Borrower's account _____

Bank: _____
Address: _____
_____
ABA Number: _____
Account Number: _____
Account Name: _____
Contact Person: _____
Phone Number
To Verify Wire Info: _____
Email address: _____

Borrower represents that the conditions precedent to the Advance set forth in the Agreement are satisfied and shall be satisfied upon the making of such Advance, including but not limited to: (i) that no event that has had or could reasonably be expected to have a Material Adverse Effect has occurred and is continuing; (ii) that the representations and warranties set forth in the Agreement are and shall be true and correct in all material respects on and as of the Advance Date with the same effect as though made on and as of such date, except to the extent such representations and warranties expressly relate to an earlier date; (iii) that Borrower is in compliance with all the terms and provisions set forth in each Loan Document on its part to be observed or performed; and (iv) that as of the Advance Date, no fact or condition exists that could (or could, with the passage of time, the giving of notice, or both) constitute an Event of Default under the Loan Documents. Borrower understands and acknowledges that Agent has the right to review the financial information supporting this representation and, based upon such review in its sole discretion, Lender may decline to fund the requested Advance.

Borrower hereby represents that Borrower's corporate status and locations have not changed since the date of the Agreement or, if the Attachment to this Advance Request is completed, are as set forth in the Attachment to this Advance Request.

[Borrower hereby authorizes Agent to deduct an amount from the proceeds of this Advance to be applied towards the payment of the Tranche 6 Facility Charge.]

Borrower agrees to notify Agent promptly before the funding of the Loan if any of the matters which have been represented above shall not be true and correct on the Borrowing Date and if Agent has received no such notice before the Advance Date then the statements set forth above shall be deemed to have been made and shall be deemed to be true and correct as of the Advance Date.

Executed as of [ ], 20[ ].

BORROWER: X4 PHARMACEUTICALS, INC.
SIGNATURE:_____
TITLE:_____

PRINT NAME:_____

ATTACHMENT TO ADVANCE REQUEST

Dated: ~~January 6~~ _____, 2023

Borrower hereby represents and warrants to Agent that the Company's current name and organizational status is as follows:

Name: X4 Pharmaceuticals, Inc.

Type of organization: Corporation

State of organization: Delaware

Organization file number: 4851982

Borrower hereby represents and warrants to Agent that the street addresses, cities, states and postal codes of its current locations are as follows:

EXHIBIT B

SECURED TERM PROMISSORY NOTE

$[ ],000,000 Advance Date: ___ __, 20[ ]

Maturity Date: _____ ___, 20[ ]

FOR VALUE RECEIVED, X4 PHARMACEUTICALS, INC., a Delaware corporation, for itself and each ~~of its Qualified Subsidiaries (the "~~other Borrower~~")~~, hereby promises to pay to the order of [Hercules Capital, Inc., a Maryland corporation], or the holder of this Note (the "Lender") at 400 Hamilton Avenue, Suite 310, Palo Alto, CA 94301 or such other place of payment as the holder of this Secured Term Promissory Note (this "Promissory Note") may specify from time to time in writing, in lawful money of the United States of America, the principal amount of [ ] Million Dollars ($[ ],000,000) or such other principal amount as Lender has advanced to Borrower, together with interest at a rate as set forth in Section 2.2(c) of the Loan Agreement based upon a year consisting of 360 days, with interest computed daily based on the actual number of days in each month.

This Promissory Note is the Note referred to in, and is executed and delivered in connection with, that certain Second Amended and Restated Loan and Security Agreement dated January 6, 2023, by and among Borrower, Hercules Capital, Inc., a Maryland corporation (the "Agent") and the several banks and other financial institutions or entities from time to time party thereto as lender (as the same may from time to time be amended, modified or supplemented in accordance with its terms, the "Loan Agreement"), and is entitled to the benefit and security of the Loan Agreement and the other Loan Documents (as defined in the Loan Agreement), to which reference is made for a statement of all of the terms and conditions thereof. All payments shall be made in accordance with the Loan Agreement. All terms defined in the Loan Agreement shall have the same definitions when used herein, unless otherwise defined herein. An Event of Default under the Loan Agreement shall constitute a default under this Promissory Note.

Borrower waives presentment and demand for payment, notice of dishonor, protest and notice of protest under the UCC or any applicable law. Borrower agrees to make all payments under this Promissory Note without setoff, recoupment or deduction and regardless of any counterclaim or defense. This Promissory Note has been negotiated and delivered to Lender and is payable in the State of California. This Promissory Note shall be governed by and construed and enforced in accordance with, the laws of the State of California, excluding any conflicts of law rules or principles that would cause the application of the laws of any other jurisdiction.

~~BORROWER~~THE COMPANY FOR ITSELF AND

~~ON BEHALF OF ITS QUALIFIED SUBSIDIARIES:~~EACH OTHER BORROWER: X4 PHARMACEUTICALS, INC.

By:_____

Title:_____

EXHIBIT C

NAME, LOCATIONS, AND OTHER INFORMATION FOR BORROWER

1. Borrower represents and warrants to Agent that

(a) the Company's current name and organizational status as of the Closing Date is as follows:

Name: X4 Pharmaceuticals, Inc.

Type of organization: Corporation

State of organization: Delaware

Organization file number: 4851982

(b) Therapeutics' current name and organizational status as of the Closing Date is as follows:

Name: X4 Therapeutics, Inc.

Type of organization: Corporation

State of organization: Delaware

Organization file number: 5568691

2. Borrower represents and warrants to Agent that for five (5) years prior to the Closing Date, Borrower did not do business under any other name or organization or form except the following:

Name:

Used during dates of:

Type of Organization:

State of organization:

Organization file Number:

Borrower's fiscal year ends on _____

Borrower's federal employer tax identification number is: _____

3. Borrower represents and warrants to Agent that its chief executive office is located at _____.

EXHIBIT D

BORROWER'S PATENTS, TRADEMARKS, COPYRIGHTS AND LICENSES

[***]

EXHIBIT E

BORROWER'S DEPOSIT ACCOUNTS AND INVESTMENT ACCOUNTS

[***]

EXHIBIT F
COMPLIANCE CERTIFICATE

Hercules Capital, Inc. (as "Agent")
400 Hamilton Avenue, Suite 310
Palo Alto, CA 94301

Reference is made to that certain Second Amended and Restated Loan and Security Agreement dated January 6, 2023 and the Loan Documents (as defined therein) entered into in connection with such Second Amended and Restated Loan and Security Agreement all as may be amended from time to time (hereinafter referred to collectively as the "Loan Agreement") by and among Hercules Capital, Inc. (the "Agent"), the several banks and other financial institutions or entities from time to time party thereto (collectively, the "Lender") and Hercules Capital, Inc., as agent for the Lender (the "Agent") and X4 Pharmaceuticals, Inc. (the "Company") as Borrower. All capitalized terms not defined herein shall have the same meaning as defined in the Loan Agreement.

The undersigned is an Officer of the Company, knowledgeable of all Company financial matters, and is authorized to provide certification of information regarding the Company; hereby certifies, in such capacity, that in accordance with the terms and conditions of the Loan Agreement, the Company is in compliance for the period ending _____ of all covenants, conditions and terms and hereby reaffirms, except as set forth below, that all representations and warranties contained therein are true and correct in all material respects on and as of the date of this Compliance Certificate with the same effect as though made on and as of such date, except to the extent such representations and warranties expressly relate to an earlier date, after giving effect in all cases to any standard(s) of materiality contained in the Loan Agreement as to such representations and warranties. Attached are the required documents supporting the above certification. The undersigned further certifies that these are prepared in accordance with GAAP (except for the absence of footnotes with respect to unaudited financial statement and subject to normal year end adjustments) and are consistent from one period to the next except as explained below.

REPORTING REQUIREMENT	REQUIRED	CHECK IF ATTACHED
Interim Financial Statements	Monthly within 30 days	
Interim Financial Statements	Quarterly within 45 days (for first 3 calendar quarters)	
Audited Financial Statements	FYE within 90 days	
Budget and forecast	At least annually within 60 days following FYE	

7.12 DEPOSIT ACCOUNTS

(A) Are all Cash balances held by Borrower held in accounts subject to an Account Control Agreement, other than Excluded Accounts? ____ Yes ____ No
(B) Is the Cash held by the Excluded Subsidiary less than $5,000,000? ____ Yes ____ No

7.21 MINIMUM CASH
(A)1 [*Prior to ~~Borrower's achievement of the Approval Milestone~~January 31, 2025*] Are aggregate Qualified Cash balances held by Borrower (excluding Security Corporation Subsidiary and the Excluded Subsidiary) in accounts subject to an Account Control Agreement in an amount greater than or equal to $20,000,000? ____ Yes ____ No
(A)2 [~~*Following Borrower's achievement of the Approval Milestone*~~*At all times on or after January 31, 2025*] Are aggregate Qualified Cash balances held by Borrower (excluding Security Corporation Subsidiary and the Excluded Subsidiary) held in accounts subject to an Account Control Agreement in an amount ~~greater than or equal $10,000,000~~equal to at least 20% of the aggregate principal amount of Term Loan Advances outstanding under the Loan Agreement? ____ Yes ____ No
(B) If No, is Security Corporation Subsidiary holding any Cash? ____ Yes ____ No
(C) If No, is the Excluded Subsidiary holding more than $5,000,000 in Cash?
____ Yes ____ No
The undersigned hereby also confirms the below disclosed accounts represent all depository accounts and securities accounts presently open in the name of each Borrower or Borrower Subsidiary/Affiliate, as applicable.
Each new account that has been opened since delivery of the previous Compliance Certificate is designated below with a "*".

		Depository AC #	Financial Institution	Account Type (Depository / Securities)	Last Month Ending Account Balance	Purpose of Account
BORROWER Name/Address:						
	1					
	2					
	3					
	4					
	5					
	6					
	7					
BORROWER SUSIDIARY / AFFILIATE COMPANY Name/Address						
	1					
	2					
	3					
	4					
	5					
	6					
	7					

7.22 PERFORMANCE COVENANT [*Commencing January 31, 2025, and thereafter*]

(A) Is Borrower's T6M Net Product Revenue, tested monthly, equal to or greater than 55% of the Net Product Revenue Forecast?

____ Yes ____ No

(B) If No, does either

(i) Borrower maintain Qualified Cash in an amount equal to or greater than ~~80~~75% of the aggregate principal amount of Term Loan Advances outstanding under this Agreement, or

(ii) both Company maintain a market capitalization of at least $~~500,000,000~~450,000,000 and also Borrower maintain Qualified Cash in an amount equal to at least ~~50~~45% of the aggregate principal amount of Term Loan Advances outstanding under this Agreement?

____ Yes ____ No

7.23 SECURITY CORPORATION INVESTMENT CONDITIONS

(A) Does the Security Corporation Subsidiary have any assets or liabilities?

____ Yes ____ No

(B) If yes, is the Security Corporation Subsidiary in compliance with the Security Corporation Investment Conditions?
____ Yes ____ No
(B) If No, Is Borrower required to maintain minimum cash under Section 7.21(a)?
____ Yes ____ No

LIST ANY EXCEPTIONS TO THE REPRESENTATIONS AND WARRANTIES.

Very Truly Yours,
X4 PHARMACEUTICALS, INC.
By: _____
Name: _____
Its: _____

EXHIBIT G

FORM OF JOINDER AGREEMENT

This Joinder Agreement (the "Joinder Agreement") is made and dated as of [ ], 20[ ], and is entered into by and between _____., a _____ corporation ("Subsidiary"), and HERCULES CAPITAL, INC., a Maryland corporation (as "Agent").

RECITALS

A. Subsidiary's Affiliate, X4 Pharmaceuticals, Inc. ("Company") has entered into that certain Second Amended and Restated Loan and Security Agreement dated as of January 6, 2023, with the several banks and other financial institutions or entities from time to time party thereto as lender (collectively, the "Lender") and the Agent, as such agreement may be amended (the "Loan Agreement"), together with the other agreements executed and delivered in connection therewith;

B. Subsidiary acknowledges and agrees that it will benefit both directly and indirectly from Company's execution of the Loan Agreement and the other agreements executed and delivered in connection therewith;

AGREEMENT

NOW THEREFORE, Subsidiary and Agent agree as follows:

1. The recitals set forth above are incorporated into and made part of this Joinder Agreement. Capitalized terms not defined herein shall have the meaning provided in the Loan Agreement.

2. By signing this Joinder Agreement, Subsidiary shall be bound by the terms and conditions of the Loan Agreement the same as if it were the Borrower (as defined in the Loan Agreement) under the Loan Agreement, mutatis mutandis, provided however, that (a) with respect to (i) Section 5.1 of the Loan Agreement, Subsidiary represents that it is an entity duly organized, legally existing and in good standing under the laws of [ ], (b) neither Agent nor Lender shall have any duties, responsibilities or obligations to Subsidiary arising under or related to the Loan Agreement or the other Loan Documents, (c) that if Subsidiary is covered by Company's insurance, Subsidiary shall not be required to maintain separate insurance or comply with the provisions of Sections 6.1 and 6.2 of the Loan Agreement, and (d) that as long as Company satisfies the requirements of Section 7.1 of the Loan Agreement, Subsidiary shall not have to provide Agent separate Financial Statements. To the extent that Agent or Lender has any duties, responsibilities or obligations arising under or related to the Loan Agreement or the other Loan Documents, those duties, responsibilities or obligations shall flow only to Company and not to Subsidiary or any other Person or entity. By way of example (and not an exclusive list): (i) Agent's providing notice to Company in accordance with the Loan Agreement or as otherwise agreed among Company, Agent and Lender shall be deemed provided to Subsidiary; (ii) a Lender's providing an Advance to Company shall be deemed an Advance to Subsidiary; and (iii) Subsidiary shall have no right to request an Advance or make any other demand on Lender.

3. Subsidiary agrees not to certificate its equity securities without Agent's prior written consent, which consent may be conditioned on the delivery of such equity securities to Agent in order to perfect Agent's security interest in such equity securities.

4. Subsidiary acknowledges that it benefits, both directly and indirectly, from the Loan Agreement, and hereby waives, for itself and on behalf on any and all successors in interest (including without limitation any assignee for the benefit of creditors, receiver, bankruptcy trustee or itself as debtor-in-possession under any bankruptcy proceeding) to the fullest extent provided by law, any and all claims, rights or defenses to the enforcement of this Joinder Agreement on the basis that (a) it failed to receive adequate consideration for the execution and delivery of this Joinder Agreement or (b) its obligations under this Joinder Agreement are avoidable as a fraudulent conveyance.

5. As security for the prompt, complete payment when due (whether on the payment dates or otherwise) of all the Secured Obligations, Subsidiary grants to Agent a security interest in all of Subsidiary's right, title, and interest in and to the Collateral.

[REMAINDER OF PAGE INTENTIONALLY LEFT BLANK]

[SIGNATURE PAGE TO JOINDER AGREEMENT]

SUBSIDIARY:

_____.

By:
Name:
Title:
Address:
Telephone: _____
email: _____
AGENT:

HERCULES CAPITAL, INC.

By:_____
Name:_____
Title: _____

Address:
400 Hamilton Ave., Suite 310
Palo Alto, CA 94301
email:
Telephone:

EXHIBIT H

ACH DEBIT AUTHORIZATION AGREEMENT

Hercules Capital, Inc.
400 Hamilton Avenue, Suite 310
Palo Alto, CA 94301

Re: Second Amended and Restated Loan and Security Agreement dated January 6, 2023 (the "Agreement") by and among X4 Pharmaceuticals, Inc. ("Borrower") and Hercules Capital, Inc., as agent ("Company") and the lenders party thereto (collectively, the "Lender")

In connection with the above referenced Agreement, the Borrower hereby authorizes the Company to initiate debit entries for (i) the periodic payments due under the Agreement and (ii) out-of-pocket legal fees and costs incurred by Agent or Lender pursuant to Section 11.11 of the Agreementto the Borrower's account indicated below. The Borrower authorizes the depository institution named below to debit to such account.

DEPOSITORY NAME	BRANCH
CITY	STATE AND ZIP CODE
TRANSIT/ABA NUMBER	ACCOUNT NUMBER

This authority will remain in full force and effect so long as any amounts are due under the Agreement.

[signature page follows]

X4 PHARMACEUTICALS, INC.
By: _____
Date: _____

[signature page to ACH Debit Authorization Agreement]

EXHIBIT I-1

FORM OF U.S. TAX COMPLIANCE CERTIFICATE

(For Foreign Lenders That Are Not Partnerships For U.S. Federal Income Tax Purposes)

Reference is hereby made to that certain Second Amended and Restated Loan and Security Agreement dated as of January 6, 2023 (as amended, supplemented or otherwise modified from time to time, the "Loan Agreement") by

and between X4 PHARMACEUTICALS, INC., a Delaware corporation, and each of its Subsidiaries (as defined in the Loan Agreement) (hereinafter collectively referred to as the "Borrower"), the several banks and other financial institutions or entities from time to time parties to the Loan Agreement (collectively, referred to as the "Lenders"), and HERCULES CAPITAL, INC., a Maryland corporation, in its capacity as administrative agent and collateral agent for itself and the Lenders (in such capacity, the "Agent").

Pursuant to the provisions of Addendum 1 of the Loan Agreement, the undersigned hereby certifies that (i) it is the sole record and beneficial owner of the Loan(s) (as well as any promissory note(s) evidencing such Loan(s)) in respect of which it is providing this certificate, (ii) it is not a "bank" within the meaning of Section 881(c)(3)(A) of the Code, (iii) it is not a "~~ten~~ 10-percent shareholder" of the Borrower within the meaning of Section 871(h)(3)(B) of the Code and (iv) it is not a "controlled foreign corporation" related to the Borrower as described in Section 881(c)(3)(C) of the Code.

The undersigned has furnished the Agent and the Borrower with a certificate of its non-U.S. Person status on IRS Form W-8BEN or IRS Form W-8BEN-E. By executing this certificate, the undersigned agrees that (1) if the information provided in this certificate changes, the undersigned shall promptly so inform the Borrower and the Agent, and (2) the undersigned shall have at all times furnished the Borrower and the Agent with a properly completed and currently effective certificate in either the calendar year in which (and in any event before) each payment is to be made to the undersigned, or in either of the two calendar years preceding such payments.

Unless otherwise defined herein, terms defined in the Loan Agreement and used herein shall have the meanings given to them in the Loan Agreement.

Date: _____ ___, 20___ [NAME OF LENDER]

By: _____
Name: _____
Title: _____

EXHIBIT I-2

FORM OF U.S. TAX COMPLIANCE CERTIFICATE

(For Foreign Participants That Are Not Partnerships For U.S. Federal Income Tax Purposes)

Reference is hereby made to that certain Second Amended and Restated Loan and Security Agreement dated as of January 6, 2023 (as amended, supplemented or otherwise modified from time to time, the "Loan Agreement") by and between X4 PHARMACEUTICALS, INC., a Delaware corporation, and each of its Subsidiaries (as defined in the Loan Agreement) (hereinafter collectively referred to as the "Borrower"), the several banks and other financial institutions or entities from time to time parties to the Loan Agreement (collectively, referred to as the "Lenders"), and HERCULES CAPITAL, INC., a Maryland corporation, in its capacity as administrative agent and collateral agent for itself and the Lenders (in such capacity, the "Agent").

Pursuant to the provisions of Addendum 1 of the Loan Agreement, the undersigned hereby certifies that (i) it is the sole record and beneficial owner of the participation in respect of which it is providing this certificate, (ii) it is not a "bank" within the meaning of Section 881(c)(3)(A) of the Code, (iii) it is not a "~~ten~~ 10-percent shareholder" of the Borrower within the meaning of Section 871(h)(3)(B) of the Code and (iv) it is not a "controlled foreign corporation" related to the Borrower as described in Section 881(c)(3)(C) of the Code.

The undersigned has furnished its participating Lender with a certificate of its non-U.S. Person status on IRS Form W-8BEN or IRS Form W-8BEN-E. By executing this certificate, the undersigned agrees that (1) if the information provided in this certificate changes, the undersigned shall promptly so inform such Lender in writing, and (2) the undersigned shall have at all times furnished such Lender with a properly completed and currently effective certificate in either the calendar year in which (and in any event before) each payment is to be made to the undersigned, or in either of the two calendar years preceding such payments.

Unless otherwise defined herein, terms defined in the Loan Agreement and used herein shall have the meanings given to them in the Loan Agreement.

Date: _____ ___, 20___ [NAME OF PARTICIPANT]

By: _____
Name: _____
Title: _____

EXHIBIT I-3

FORM OF U.S. TAX COMPLIANCE CERTIFICATE

(For Foreign Participants That Are Partnerships For U.S. Federal Income Tax Purposes)

Reference is hereby made to that certain Second Amended and Restated Loan and Security Agreement dated as of January 6, 2023 (as amended, supplemented or otherwise modified from time to time, the "Loan Agreement") by and between X4 PHARMACEUTICALS, INC., a Delaware corporation, and each of its Subsidiaries (as defined in the Loan Agreement) (hereinafter collectively referred to as the "Borrower"), the several banks and other financial institutions or entities from time to time parties to the Loan Agreement (collectively, referred to as the "Lenders"), and HERCULES CAPITAL, INC., a Maryland corporation, in its capacity as administrative agent and collateral agent for itself and the Lenders (in such capacity, the "Agent").

Pursuant to the provisions of Addendum 1 of the Loan Agreement, the undersigned hereby certifies that (i) it is the sole record owner of the participation in respect of which it is providing this certificate, (ii) its direct or indirect partners/members are the sole beneficial owners of such participation, (iii) with respect such participation, neither the undersigned nor any of its direct or indirect partners/members is a "bank" extending credit pursuant to a loan agreement entered into in the ordinary course of its trade or business within the meaning of Section 881(c)(3)(A) of the Code, (iv) none of its direct or indirect partners/members is a "~~ten~~ 10-percent shareholder" of the Borrower within the

meaning of Section 871(h)(3)(B) of the Code and (v) none of its direct or indirect partners/members is a "controlled foreign corporation" related to the Borrower as described in Section 881(c)(3)(C) of the Code.

The undersigned has furnished its participating Lender with IRS Form W-8IMY accompanied by one of the following forms from each of its partners/members that is claiming the portfolio interest exemption: (i) an IRS Form W-8BEN or IRS Form W-8BEN-E or (ii) an IRS Form W-8IMY accompanied by an IRS Form W-8BEN or IRS Form W-8BEN-E from each of such partner's/member's beneficial owners that is claiming the portfolio interest exemption. By executing this certificate, the undersigned agrees that (1) if the information provided in this certificate changes, the undersigned shall promptly so inform such Lender and (2) the undersigned shall have at all times furnished such Lender with a properly completed and currently effective certificate in either the calendar year in which (and in any event before) each payment is to be made to the undersigned, or in either of the two calendar years preceding such payments.

Unless otherwise defined herein, terms defined in the Loan Agreement and used herein shall have the meanings given to them in the Loan Agreement.

Date: _____ ___, 20___ [NAME OF PARTICIPANT]

By: _____
Name: _____
Title: _____

EXHIBIT I-4

FORM OF U.S. TAX COMPLIANCE CERTIFICATE

(For Foreign Lenders That Are Partnerships For U.S. Federal Income Tax Purposes)

Reference is hereby made to that certain Second Amended and Restated Loan and Security Agreement dated as of January 6, 2023 (as amended, supplemented or otherwise modified from time to time, the "Loan Agreement") by and between X4 PHARMACEUTICALS, INC., a Delaware corporation, and each of its Subsidiaries (as defined in the Loan Agreement) (hereinafter collectively referred to as the "Borrower"), the several banks and other financial institutions or entities from time to time parties to the Loan Agreement (collectively, referred to as the "Lenders"), and HERCULES CAPITAL, INC., a Maryland corporation, in its capacity as administrative agent and collateral agent for itself and the Lenders (in such capacity, the "Agent").

Pursuant to the provisions of Addendum 1 of the Loan Agreement, the undersigned hereby certifies that (i) it is the sole record owner of the Loan(s) (as well as any promissory note(s) evidencing such Loan(s)) in respect of which it is providing this certificate, (ii) its direct or indirect partners/members are the sole beneficial owners of such Loan(s) (as well as any promissory note(s) evidencing such Loan(s)), (iii) with respect to the extension of credit pursuant to this Loan Agreement or any other Loan Document, neither the undersigned nor any of its direct or indirect partners/members is a "bank" extending credit pursuant to a loan agreement entered into in the ordinary course of its trade or business within the meaning of Section 881(c)(3)(A) of the Code, (iv) none of its direct or indirect

partners/members is a "~~ten~~ 10-percent shareholder" of the Borrower within the meaning of Section 871(h)(3)(B) of the Code and (v) none of its direct or indirect partners/members is a "controlled foreign corporation" related to the Borrower as described in Section 881(c)(3)(C) of the Code.

The undersigned has furnished the Agent and the Borrower with IRS Form W-8IMY accompanied by one of the following forms from each of its partners/members that is claiming the portfolio interest exemption: (i) an IRS Form W-8BEN or IRS Form W-8BEN-E or (ii) an IRS Form W-8IMY accompanied by an IRS Form W-8BEN or IRS Form W-8BEN-E from each of such partner's/member's beneficial owners that is claiming the portfolio interest exemption. By executing this certificate, the undersigned agrees that (1) if the information provided in this certificate changes, the undersigned shall promptly so inform the Borrower and the Agent, and (2) the undersigned shall have at all times furnished the Borrower and the Agent with a properly completed and currently effective certificate in either the calendar year in which (and in any event before) each payment is to be made to the undersigned, or in either of the two calendar years preceding such payments.

Unless otherwise defined herein, terms defined in the Loan Agreement and used herein shall have the meanings given to them in the Loan Agreement.

Date: _____ ___, 20___ [NAME OF LENDER]

By: _____
Name: _____
Title: _____

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SCHEDULES TO

SECOND AMENDED AND RESTATED
LOAN AND SECURITY AGREEMENT

dated as of January 6, 2023
(other than Schedule 1.1, which is dated as of the First Amendment Effective Date)

SCHEDULE 1

SUBSIDIARIES

[***]

SCHEDULE 1.1

COMMITMENTS

[***]

SCHEDULE 1A

EXISTING PERMITTED INDEBTEDNESS

[***]

SCHEDULE 1B

EXISTING PERMITTED INVESTMENTS

[***]

SCHEDULE 1C

EXISTING PERMITTED LIENS

[***]

SCHEDULE 3.2

EXCLUDED COLLATERAL (SANOFI)

[***]

SCHEDULE 5.3

CONSENTS, ETC.

[***]

SCHEDULE 5.8

TAX MATTERS

[***]

SCHEDULE 5.9

INTELLECTUAL PROPERTY CLAIMS

[***]

SCHEDULE 5.10

INTELLECTUAL PROPERTY

[***]

SCHEDULE 5.11

BORROWER PRODUCTS

[***]

SCHEDULE 5.13

EMPLOYEE LOANS

[***]

SCHEDULE 5.14

CAPITALIZATION

[***]

SCHEDULE 7.24

POST-CLOSING ITEMS

[***]

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: November 9, 2023 May 7, 2024

/s/ Paula Ragan, Ph.D.

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

Exhibit 31.2

CERTIFICATION

I, Adam S. Mostafa, certify that:

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

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

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

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

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

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

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

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

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

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

and

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

Date: November 9, 2023 May 7, 2024

/s/ Adam Mostafa

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

Exhibit 32.1

CERTIFICATION

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

1. The Company's Quarterly Report on Form 10-Q for the period ended September 30, 2023 March 31, 2024, to which this Certification is attached as Exhibit 32.1 (the "Quarterly Report"), fully complies with the requirements of Section 13(a) or Section 15(d) of the Exchange Act; and

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

In Witness Whereof, the undersigned have set their hands hereto as of the 9th 7th day of November, 2023. May, 2024.

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

Paula Ragan, Ph.D. Adam S. Mostafa

Chief Executive Officer Chief Financial Officer

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

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