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, 2020

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)

955 Massachusetts Avenue, 4th Floor
Cambridge, Massachusetts 02139
(Former name, former address and former year, if changed since last report)

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock	**XFOR**	**The Nasdaq Stock Market LLC**

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 2, 2020, the registrant had 16,286,645 shares of common stock 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, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. 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, although not all forward looking statements contain these identifying words. Our forward-looking statements are based on a series of expectations, assumptions, estimates and projections about our company, are not guarantees of future results or performance and involve substantial risks and uncertainty. We may not actually achieve the plans, intentions or expectations disclosed in these forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in these forward-looking statements. These forward-looking statements are subject to a number of known and unknown risks, uncertainties and assumptions, including risks described in the section titled "Risk Factors" and elsewhere in this report, regarding, among other things:

- the timing, progress and reporting of results of our current trials of X4P-001 (mavorixafor), including our global Phase 3 clinical trial in patients with Warts, Hypogammaglobulinemia, Infections, and Myelokathexis, or WHIM, syndrome, our Phase 1b clinical trial in combination with ibrutinib in patients with Waldenström's macroglobulinemia, and our Phase 1b clinical trial as monotherapy in patients with Severe Congenital Neutropenia, or SCN;
- the initiation, timing, progress and results of our current and future preclinical studies and clinical trials of X4P-002 and X4P-003 or any of our other product candidates or our research and development programs that we pursue;
- our expectations regarding the impact of the ongoing coronavirus disease 2019, or COVID-19, pandemic, included the expected duration of disruption and immediate and long-term impact and effect on our business and operations;
- the diversion of healthcare resources away from the conduct of clinical trials as a result of the ongoing COVID-19 pandemic, including the diversion of hospitals serving as our clinical trial sites and hospital staff or independent physicians supporting the conduct of our clinical trials;
- the interruption of key clinical trial activities, such as clinical trial site monitoring, due to limitations on travel, quarantines or social distancing protocols imposed or recommended by federal or state governments, employers and others in connection with the ongoing COVID-19 pandemic;
- the potential benefits that may be derived from any of our product candidates;
- the timing of and our ability to obtain and maintain regulatory approval of our existing product candidates or any product candidates that we may develop in the future, and any related restrictions, limitations, or warnings in the label of any approved product candidates;
- our plans to research, develop, manufacture and commercialize our product candidates;
- the timing of our regulatory filings for our product candidates, along with regulatory developments in the United States and other foreign countries;
- the size and growth potential of the markets for our product candidates, if approved, and the rate and degree of market acceptance of our product candidates, including reimbursement that may be received from payors;
- the benefits of U.S. Food and Drug Administration, or FDA, and European Commission designations such as, 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;

- 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 raise additional capital; and
- our strategies, prospects, plans, expectations or objectives.

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

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, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 88,812	$ 126,184
Research and development incentive receivable	629	1,998
Prepaid expenses and other current assets	4,472	1,096
Total current assets	93,913	129,278
Property and equipment, net	1,334	403
Goodwill	27,109	27,109
Right-of-use assets	8,222	1,959
Other assets	2,548	1,949
Total assets	$ 133,126	$ 160,698
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 3,891	$ 2,088
Accrued expenses	8,532	6,461
Current portion of lease liability	756	898
Total current liabilities	13,179	9,447
Long-term debt, including accretion, net of discount	25,537	20,097
Lease liabilities	4,677	1,918
Other liabilities	25	16
Total liabilities	43,418	31,478
Commitments and contingencies (Note 9)		
Stockholders' equity:		
Common stock, $0.001 par value, 125,000,000 and 33,333,333 shares authorized as of September 30, 2020 and December 31, 2019, respectively; 16,286,645 and 16,128,862 shares issued and outstanding as of September 30, 2020 and December 31, 2019, respectively	16	16
Additional paid-in capital	265,586	261,367
Accumulated other comprehensive loss	(119)	(119)
Accumulated deficit	(175,775)	(132,044)
Total stockholders' equity	89,708	129,220
Total liabilities and stockholders' equity	$ 133,126	$ 160,698

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 per share amounts)

(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
License revenue	$ —	$ —	$ 3,000	$ —
Operating expenses:				
Research and development	11,381	8,589	29,634	23,098
General and administrative	5,599	4,383	15,585	13,726
Loss on transfer of non-financial assets	—	4,004	—	4,004
Total operating expenses	16,980	16,976	45,219	40,828
Loss from operations	(16,980)	(16,976)	(42,219)	(40,828)
Other income (expense):				
Interest income	—	464	272	927
Interest expense	(697)	(688)	(1,968)	(1,599)
Change in fair value of preferred stock warrant liability	—	—	—	(288)
Change in fair value of derivative liability	—	—	—	183
Other income	228	52	494	201
Loss on extinguishment of debt	—	(566)	(162)	(566)
Total other expense, net	(469)	(738)	(1,364)	(1,142)
Loss before provision for income taxes	(17,449)	(17,714)	(43,583)	(41,970)
Provision for income taxes	—	—	148	—
Net loss	(17,449)	(17,714)	(43,731)	(41,970)
Accruing dividends on Series A convertible preferred stock	—	—	—	(592)
Net loss attributable to common stockholders	$ (17,449)	$ (17,714)	$ (43,731)	$ (42,562)
Net loss per share attributable to common stockholders—basic and diluted	$ (0.87)	$ (1.22)	$ (2.18)	$ (4.31)
Weighted average common shares outstanding—basic and diluted	20,085	14,562	20,035	9,866

Net loss	$ (17,449)	$ (17,714)	$ (43,731)	$ (41,970)
Currency translation adjustments	—	(77)	—	(119)
Total comprehensive loss	$ (17,449)	$ (17,791)	$ (43,731)	$ (42,089)

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

X4 PHARMACEUTICALS, INC.

CONDENSED CONSOLIDATED STATEMENT OF CONVERTIBLE PREFERRED STOCK, REDEEMABLE COMMON STOCK AND STOCKHOLDERS' EQUITY (DEFICIT)

(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, 2019	**16,128,862**	**$ 16**	**$ 261,367**	**$ (119)**	**$ (132,044)**	**$ 129,220**
Exercise of stock options	13,006	—	96			**96**
Stock-based compensation expense			613			**613**
Net loss					(11,138)	**(11,138)**
Balance at March 31, 2020	16,141,868	16	262,076	(119)	(143,182)	**118,791**
Exercise of stock options	407	—	2			**2**
Issuance of shares under employee stock purchase plan	10,057	—	76			**76**
Vesting of restricted stock units, less shares withheld and retired to satisfy tax obligations	23,822		(14)			**(14)**
Stock-based compensation expense			1,173			**1,173**
Net loss					(15,144)	**(15,144)**
Balance at June 30, 2020	16,176,154	16	263,313	(119)	(158,326)	**104,884**
Exercise of stock options	4,276		29			**29**
Vesting of restricted stock units, less shares withheld and retired to satisfy tax obligations	106,215					**0**
Stock-based compensation expense			2,244			**2,244**
Net loss					(17,449)	**(17,449)**
Balance at September 30, 2020	**16,286,645**	**$ 16**	**$ 265,586**	**$ (119)**	**$ (175,775)**	**$ 89,708**

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

X4 PHARMACEUTICALS, INC.

CONDENSED CONSOLIDATED STATEMENT OF CONVERTIBLE PREFERRED STOCK, REDEEMABLE COMMON STOCK AND STOCKHOLDERS' EQUITY (DEFICIT)

(In thousands, except share amounts)

(Unaudited)

	Series Seed, A and B Convertible Preferred		Redeemable Common Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Stockholders' (Deficit)
	Shares	Amount	Shares	Amount	Shares	Amount				
Balance at December 31, 2018	**40,079,567**	**$ 64,675**	**107,364**	**$ 734**	**351,652**	**—**	**$ 2,151**	**—**	**$ (79,237)**	**$ (77,086)**
Conversion of redeemable common stock into common stock			(107,364)	(734)	107,364	—	733			**733**
Conversion of convertible preferred shares into common stock	(40,079,567)	(64,675)			3,808,430	4	64,671			**64,675**
Exchange of common stock in connection with Merger					2,440,582	2	45,539			**45,541**
Fair value of replacement equity awards							817			**817**
Reclassification of warrant liability to permanent equity							5,235			**5,235**
Exercise of stock options					16,483		113			**113**
Stock-based compensation							262			**262**
Currency translation adjustment								23		**23**
Net loss									(10,873)	**(10,873)**
Balance at March 31, 2019	—	—	—	—	6,724,511	6	119,521	23	(90,110)	**29,440**
Issuance of common stock and prefunded warrants for the purchase of common stock, net of issuance costs of $931					5,670,000	6	79,291			**79,297**
Exercise of stock options					700		5			**5**
Exercise of warrants					33,846		440			**440**
Stock-based compensation							433			**433**
Currency translation adjustment								(65)		**(65)**
Net loss									(13,383)	**(13,383)**
Balance at June 30, 2019	—	—	—	—	12,429,057	12	199,690	(42)	(103,493)	**96,167**
Exercise of stock options					7,148	—	40			**40**
Stock-based compensation expense							691			**691**
Currency translation adjustment								(77)		**(77)**
Net loss									(17,714)	**(17,714)**
Balance at September 30, 2019	**—**	**—**	**—**	**—**	**12,436,205**	**$ 12**	**$ 200,421**	**$ (119)**	**$ (121,207)**	**$ 79,107**

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,	
	2020	**2019**
Cash flows from operating activities:		
Net loss	$ (43,731)	$ (41,970)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	4,030	1,386
Depreciation and amortization expense	248	71
Loss on transfer of non-financial assets	—	4,004
Non-cash lease expense	588	416
Accretion of debt discount	389	584
Loss on extinguishment of debt	162	566
Change in fair value of preferred stock warrant liability	—	288
Change in fair value of derivative liability	—	(183)
Other	(219)	—
Changes in operating assets and liabilities:		
Prepaid expenses, other current assets and research and development incentive receivable	(2,420)	290
Accounts payable	121	(3,131)
Accrued expenses	1,722	(595)
Lease liabilities	(904)	(540)
Operating lease right-of-use asset, net of non-cash portion	(1,300)	—
Net cash used in operating activities	(41,314)	(38,814)
Cash flows from investing activities:		
Cash, cash equivalents and restricted cash acquired in connection with the Merger	—	26,406
Proceeds from transfer of non-financial assets	—	896
Acquisition of property, equipment and intangible assets	(1,060)	(91)
Net cash (used in) provided by investing activities	(1,060)	27,211
Cash flows from financing activities:		
Proceeds from exercise of stock options and warrants	468	605
Employee taxes paid related to net share settlement of vested restricted stock units	(278)	—
Proceeds from borrowings under loan and security agreements, net of issuance costs	4,888	9,849
Proceeds from sale of common stock and warrants, net of issuance costs	(313)	79,291
Repayments of borrowings under loan and security agreement	—	(9,368)
Net cash provided by financing activities	4,765	80,377
Effect of exchange rate changes on cash, cash equivalents and restricted cash	240	(266)
Net (decrease) increase in cash, cash equivalents and restricted cash	(37,369)	68,508
Cash, cash equivalents and restricted cash at beginning of period	128,086	8,498
Cash, cash equivalents and restricted cash at end of period	$ 90,717	$ 77,006
Supplemental disclosure of non-cash investing and financing activities:		
Acquisition of property, equipment and right-of-use assets included in accounts payable and accrued expenses	$ 2,052	$ 38
Acquisition of right-of-use asset financed by lease liabilities	$ 4,646	$ —
Issuance costs not yet paid	$ 178	$ —
Conversion of convertible preferred stock into common stock	$ —	$ 64,675
Conversion of redeemable common stock into common stock	$ —	$ 734
Conversion of convertible preferred stock warrants into common stock warrants	$ —	$ 5,235
Fair value of net assets acquired in the Merger	$ —	$ 46,358

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

1. Nature of the Business and Basis of Presentation

X4 Pharmaceuticals, Inc. (together with its subsidiaries, the "Company") is a late-stage clinical biopharmaceutical company focused on the research, development and commercialization of novel therapeutics for the treatment of rare diseases. The Company's lead product candidate, mavorixafor, is a potential first-in-class, once-daily, oral inhibitor of CXCR4 and is currently in a Phase 3 clinical trial for the treatment of Warts, Hypogammaglobulinemia, Infections, and Myelokathexis ("WHIM") syndrome, a rare, inherited, primary immunodeficiency disease caused by genetic mutations in the CXCR4 receptor gene. The Company is also conducting a 14-day, proof-of-concept Phase 1b clinical trial of mavorixafor in patients with severe congenital neutropenia ("SCN") and a Phase 1b clinical trial of mavorixafor in combination with ibrutinib in Waldenström's macroglobulinemia ("Waldenström's").

***Liquidity*—**As of September 30, 2020, the Company had $88.8 million of cash and cash equivalents and an accumulated deficit of $175.8 million. As of the issuance date of these condensed consolidated financial statements, the Company expects that its cash and cash equivalents will be sufficient to fund its forecasted operating expenses, capital expenditure requirements and debt service payments for at least the next twelve months from the issuance date of these condensed consolidated financial statements.

Since its inception, the Company has incurred significant operating losses and negative cash flows from operations. The Company has not yet commercialized a product and does not expect to generate revenue from the commercial sale of any products for several years, if at all. The Company expects that its research and development and general and administrative expenses will continue to increase and, as a result, will need additional capital to fund its future operations, which it may raise through a combination of equity offerings, debt financings, other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements. The Company has funded its operations to date primarily with proceeds from sales of common stock, warrants and prefunded warrants for the purchase of shares of preferred stock and shares of common stock, sales of preferred stock, proceeds from the issuance of convertible debt and borrowings under loan and security agreements.

If the Company is unable to obtain future funding when needed, the Company may be forced to delay, reduce or eliminate some or all of its research and development programs, product portfolio expansion or pre-commercialization efforts, which could adversely affect its business prospects, or the Company may be unable to continue operations. There is no assurance that the Company will be successful in obtaining sufficient funding on terms acceptable to the Company to fund continuing operations, if at all.

***Impact of the COVID-19 Pandemic*—** The impact of the COVID-19 pandemic has been and is expected to continue to be extensive in many aspects of society, which has resulted in and will likely continue to result in significant disruptions to the global economy, as well as businesses and capital markets around the world. Impacts to the Company's business have included temporary closures or postponements of activation of its clinical trial sites or facilities, disruptions or restrictions on its employees' ability to travel, disruptions to or delays in ongoing clinical trials, including patient enrollment at a slower pace than initially projected and the diversion of healthcare resources away from the conduct of the Company's clinical trials as a result of the ongoing COVID-19 pandemic, including the diversion of hospitals serving as the Company's clinical trial sites and hospital staff supporting the conduct of the Company's clinical trials.

***Merger with Arsanis*—** On November 26, 2018, Arsanis, Inc., a publicly held Delaware corporation ("Arsanis"), Artemis AC Corp., a Delaware corporation and a wholly-owned subsidiary of Arsanis ("Merger Sub"), and X4 Therapeutics, Inc. ("X4") entered into an Agreement and Plan of Merger, as amended on December 20, 2018 and March 8, 2019 (the "Merger Agreement"), pursuant to which the Merger Sub merged with and into X4, with X4 surviving the merger as a wholly-owned subsidiary of Arsanis. The transactions described in the foregoing sentence may be referred to in these condensed consolidated financial statements as "the Merger."

On March 13, 2019, Arsanis, X4 and Merger Sub completed the Merger pursuant to the terms of the Merger Agreement. Pursuant to the terms of the Merger Agreement, each outstanding share of X4's common stock and preferred stock was exchanged for 0.5702 shares of Arsanis' common stock (the "Exchange Ratio"). In addition, all outstanding options exercisable for common stock and warrants exercisable for convertible preferred stock of X4 became options and warrants exercisable for the same number of shares of common stock of Arsanis multiplied by the Exchange Ratio. In connection with the Merger, X4 changed its name to X4 Therapeutics, Inc. Following the closing of the Merger, X4 Therapeutics, Inc. became a wholly-owned

subsidiary of the Company, which changed its name to X4 Pharmaceuticals, Inc. As used herein, the words “the Company” refers to, for periods following the Merger, X4 Pharmaceuticals, Inc. (formerly Arsanis, Inc.), together with is direct and indirect subsidiaries, and for periods prior to the Merger, X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals, Inc.), and its direct and indirect subsidiaries, as applicable.

2. Summary of Significant Accounting Policies

***Significant Accounting Policies*—**The Company’s significant accounting policies are disclosed in the audited consolidated financial statements in the Company’s Annual Report on Form 10-K for the year ended December 31, 2019 and the notes thereto filed with the U.S. Securities and Exchange Commission (“SEC”) on March 12, 2020. Since the date of those consolidated financial statements, there have been no material changes to the Company's significant accounting policies other than as listed below.

***Risks and Uncertainties*—**With the global spread of the ongoing COVID-19 pandemic in the first half of 2020, the Company has implemented and continues to evaluate business continuity measures designed to address and mitigate the impact of the COVID-19 pandemic on its business. The COVID-19 pandemic has impacted and is expected to continue to impact the clinical development timelines for certain of the Company's clinical programs. The extent to which the COVID-19 pandemic impacts the Company’s business, its clinical development and regulatory efforts, its corporate development objectives and the value of and market for its common stock, will depend on future developments that are highly uncertain and cannot be predicted with confidence at this time, such as the ultimate duration of the pandemic, travel restrictions, quarantines, social distancing and business closure requirements in the United States, Europe and other countries, and the effectiveness of actions taken globally to contain and treat the disease. While the Company is experiencing limited financial impacts at this time, the duration and intensity of these impacts and resulting disruption to the Company's operations is uncertain and the Company will continue to assess the financial impact.

In addition, the Company is subject to other challenges and risks specific to its business and its ability to execute on its business plan and strategy, as well as risks and uncertainties common to companies in the biotechnology industry with research and development operations, including, without limitation, risks and uncertainties associated with: obtaining regulatory approval of its product candidates; delays or problems in obtaining clinical supply, loss of single source suppliers or failure to comply with manufacturing regulations; identifying, acquiring or in-licensing additional products or product candidates; product development and the inherent uncertainty of clinical success; and the challenges of protecting and enhancing its intellectual property rights; and the challenges of complying with applicable regulatory requirements. In addition, to the extent the ongoing COVID-19 pandemic adversely affects the Company’s business and results of operations, it is expected also to have the effect of heightening many of the other risks and uncertainties discussed above.

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

***Unaudited Interim Condensed Consolidated Financial Statements*—** The condensed consolidated balance sheet at December 31, 2019 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 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, 2019 included in the Company’s Annual Report on Form 10-K for the year ended December 31, 2019 filed with the SEC on March 12, 2020. 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 cash flows have been made. The results of operations for the three and nine months ended September 30, 2020 are not necessarily indicative of the results of operations that may be expected for the year ending December 31, 2020.

Use of Estimates— The preparation of the Company's condensed consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the condensed consolidated financial statements, and the reported amounts of expenses during the reporting period. Significant estimates and assumptions reflected in these condensed consolidated financial statements include, but are not limited to, the accrual of research and development expenses, the impairment or lack of impairment of long-lived assets including operating lease right-of-use assets and goodwill, and the constraint of variable consideration from contracts with customers. 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. The COVID-19 pandemic has impacted and is expected to continue to impact the clinical development timelines for certain of the Company's clinical programs. 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 condensed consolidated financial statements.

Cash and Cash Equivalents— The Company considers all highly liquid investments with maturities of three months or less at the date of purchase to be cash equivalents. Cash equivalents consisted of money market funds as of September 30, 2020 and December 31, 2019.

Restricted Cash—

(in thousands)	As of September 30, 2020	As of December 31, 2019
Letter of credit security: Cambridge lease	$ 264	$ 264
Letter of credit security: Waltham lease	250	250
Letter of credit security: Vienna Austria lease	97	94
Letter of credit security: Boston lease	1,144	1,144
Corporate credit card collateral	150	150
Total restricted cash (non-current)	$ 1,905	$ 1,902

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 following table provides a reconciliation of cash, cash equivalents, and restricted cash reported within the condensed consolidated balance sheets to the sum of the total of amounts shown in the Company's condensed consolidated statements of cash flows as of September 30, 2020, December 31, 2019, September 30, 2019 and December 31, 2018:

(in thousands)	September 30, 2020	December 31, 2019	September 30, 2019	December 31, 2018
Cash and cash equivalents	$ 88,812	$ 126,184	$ 76,251	$ 8,134
Restricted cash, non-current	1,905	1,902	755	364
Total cash, cash equivalents and restricted cash	$ 90,717	$ 128,086	$ 77,006	$ 8,498

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.

Given the declaration of the COVID-19 pandemic and associated volatility reflected in the global financial markets and uncertain impact on the Company's operations, management conducted an interim test of its goodwill as of March 31, 2020 and determined that goodwill was not impaired as of that date. There were no additional triggering events during the nine months ended September 30, 2020 that necessitated an interim impairment test of goodwill.

Recently Adopted Accounting Pronouncements

In August 2018, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") 2018-15, *Intangibles-Goodwill and Other-Internal-Use Software (Subtopic 350-40): Customer's Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That Is a Service Contract*, ("ASU 2018-15"). The amendments in this update align the requirements for capitalizing implementation costs incurred in a hosting arrangement that is a service contract with the requirements for capitalizing implementation costs incurred to develop or obtain internal-use software (and hosting arrangements that include an internal-use software license). The accounting for the service element of a hosting arrangement that is a service contract is not affected by the amendments in this update. The new standard was effective beginning January 1, 2020 and was adopted by the Company on that date. The adoption of ASU 2018-15 did not have an impact on the Company's consolidated financial statements and related disclosures.

In August 2018, the FASB issued ASU No. 2018-13, *Fair Value Measurement (Topic 820): Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement* ("ASU 2018-13"), which removes, adds and modifies certain disclosure requirements for fair value measurements in Topic 820. The Company will no longer be required to disclose the amount of and reasons for transfers between Level 1 and Level 2 of the fair value hierarchy as well as the valuation processes of Level 3 fair value measurements. The Company will be required to provide additional disclosure related to the changes in unrealized gains and losses included in other comprehensive loss for recurring Level 3 fair value measurements and the range and weighted average of assumptions used to develop significant unobservable inputs for Level 3 fair value measurements. ASU 2018-13 is effective for the Company on January 1, 2020 and was adopted on that date. The adoption of ASU 2018-13 did not have a significant impact on the Company's consolidated financial statements and related disclosures.

Recently Issued Accounting Standards Not Yet Adopted

In December 2019, the FASB issued ASU 2019-12, *Simplifying the Accounting for Income Taxes* ("ASU 2019-12"). ASU 2019-12 simplifies the accounting for income taxes, including the methodology for calculating income taxes in an interim period and the recognition of deferred tax liabilities for outside basis differences. ASU 2019-12 is effective for fiscal years beginning after December 15, 2020, including interim periods within those fiscal years. The Company does not anticipate that the adoption of ASU 2019-12 will have a material impact on its consolidated financial statements and related disclosures.

In June 2016, the FASB issued ASU 2016-13, *Credit Losses (Topic 326) Measurement of Credit Losses on Financial Instruments* ("ASU 2016-13"), as amended. ASU 2016-13 requires that financial assets measured at amortized cost, such as trade receivables, be presented net of expected credit losses, which may be estimated based on relevant information such as historical experience, current conditions, and future expectation for each pool of similar financial asset. The new guidance requires enhanced disclosures related to trade receivables and associated credit losses. In accordance with ASU 2019-10, *Financial Instruments-Credit Losses (Topic 326), Derivative and Hedging (Topic 815), and Leases (Topic 842)- Effective Dates*, as the Company meets the definition of a "smaller reporting company", the Company has elected to defer the adoption of ASU 2016-13 until January 1, 2023. The Company expects that the adoption of ASU 2016-13 may accelerate the timing and could increase the level of credit loss expense in the consolidated statement of operations and will likely require an increased level of disclosure in the notes to the consolidated financial statements.

3. License, Collaboration and Funding Agreements

There were no material modifications of the Company's license, collaboration or funding agreements during the three months ended September 30, 2020.

Research and Development Incentive Program

The Company participates in a research and development incentive program provided by the Austrian government whereby the Company is entitled to reimbursement by the Austrian government for a percentage of qualifying research and development expenses incurred by the Company's subsidiary in Austria. Under the program, the reimbursement rate for qualifying research and development expenses incurred by the Company through its subsidiary in Austria is 14% for the years ended December 31, 2020 and 2019. As of September 30, 2020, the amount due under the program is $0.6 million, which amount was included in research and development incentive receivable in the condensed consolidated balance sheet. During the three and nine months ended September 30, 2020, the Company recorded $97 thousand and $267 thousand of income, respectively, related to the program within the condensed consolidated statements of operations as "other income".

Abbisko Agreement

In July 2019, the Company entered into a license agreement, (the "Abbisko Agreement") with Abbisko Therapeutics Co., Ltd., ("Abbisko"). Under the terms of the Abbisko Agreement, the Company granted Abbisko the exclusive right to develop, manufacture and commercialize mavorixafor in mainland China, Taiwan, Hong Kong and Macau, the ("Abbisko Territory"). The agreement provides Abbisko with the exclusive rights in the Abbisko Territory to develop and commercialize mavorixafor in combination with checkpoint inhibitors or other agents in multiple oncology indications. The Company retains the full rest-of-world rights to develop and commercialize mavorixafor outside of the Abbisko Territory for all indications and the Company has the ability to utilize data generated pursuant to the Abbisko collaboration for rest-of-world development. Assuming mavorixafor is developed by Abbisko in six indications, the Company would be entitled to milestone payments of up to $208 million, which will vary based on the ultimate sales, if any, of the approved licensed products. In addition, upon commercialization of mavorixafor in the Abbisko Territory, the Company is eligible to receive a tiered royalty, with a percentage range in the low double-digits, on net sales of approved licensed products. Abbisko is obligated to use commercially reasonable efforts to develop and commercialize mavorixafor in the Abbisko Territory.

Following the closing of a qualified financing (as such term is defined in the Abbisko Agreement), Abbisko was required to pay the Company a one-time, non-refundable, non-creditable financial milestone payment of $3.0 million. Abbisko achieved such qualified financing in March 2020 and, as a result, the Company was eligible to receive the milestone payment, which was received by the Company in April 2020. The Company is also eligible to receive potential development and regulatory milestone payments, which vary based on the number of indications developed, and potential commercial milestone payments based on annual net sales of mavorixafor-based licensed products.

Upon entering into the Abbisko Agreement, the Company evaluated the agreement under Accounting Standards Codification Topic 606 ("ASC 606") and determined the agreement contained a single performance obligation related to the exclusive license to develop and commercialize mavorixafor and the transfer of know-how that was satisfied at the inception of the arrangement. The transaction price related to the transfer of the license and know-how was fully constrained at the inception of the arrangement and the Company ascribed no transaction price to the development, regulatory and commercial milestones under the "most-likely-amount" method. The Company concluded that any consideration related to the initial transfer of the license and know-how will be recognized when it is probable that Abbisko will achieve the related financial milestone and other operational milestones. As a result of Abbisko's achievement of the qualified financing, the Company reversed the constraint related to this milestone and recognized $3.0 million of license revenue during the three months ended March 31, 2020.

As of September 30, 2020, Abbisko has not achieved any additional development or regulatory operational milestones, other than as noted above and, therefore, the Company continues to fully constrain the value of any future milestone payments. The Company will continue to re-evaluate the transaction price and associated constrained amounts in each reporting period and as uncertain events are resolved or other changes in circumstances occur.

4. Fair Value of Financial Assets and Liabilities

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

	Fair Value Measurements as of September 30, 2020 Using:			
(in thousands)	**Level 1**	**Level 2**	**Level 3**	**Total**
Assets:				
Cash equivalents—money market funds	$ 46,815	$ 1,843	$ —	$ 48,658
	$ 46,815	$ 1,843	$ —	$ 48,658

Liabilities: None

	Fair Value Measurements as of December 31, 2019 Using:			
(in thousands)	**Level 1**	**Level 2**	**Level 3**	**Total**
Assets:				
Cash equivalents—money market funds	$ 23,638	$ 39,999	$ —	$ 63,637
	$ 23,638	$ 39,999	$ —	$ 63,637

Liabilities: None

The Company's cash equivalents consisted of money market funds invested in U.S. Treasury securities. The money market funds were valued based on reported market pricing for the identical assets, which represents a Level 1 measurement, or by using inputs observable in active markets for similar securities, which represents a Level 2 measurement.

During the nine months ended September 30, 2020, the Company refinanced its Amended and Restated Loan and Security Agreement (as defined in Note 7) with Hercules Capital Inc. ("Hercules"). The debt refinancing was deemed to be an extinguishment of the June 2019 Amended and Restated Loan and Security Agreement (the "previous debt") in exchange for the Amended Loan Agreement (the "new debt") as further described in Note 7. The loss on extinguishment was calculated as the difference between the carrying amount of the previous debt and the fair value of the new debt, which is a non-recurring Level 3 measure. The fair value of the new debt was calculated based on the cash flows of the new debt, including end-of-term payments, interest payments, principal payments and lender fees, discounted to present value using the appropriate market rate. The market rate is a significant unobservable input. The Company developed a range of market rates in reference to the Company's recent borrowing activities with this lender, which are deemed to be reflective of the market rate. The Company considered a range from 10.5% to 12.5% and determined that 11.5% represented a reasonable rate for purposes of determining the fair value of the new debt, which was $25.3 million.

5. Property and Equipment, Net

Property and equipment, net consisted of the following:

(in thousands)	**September 30, 2020**	**December 31, 2019**
Leasehold improvements	$ 216	$ 299
Furniture and fixtures	913	139
Computer equipment	47	37
Software	33	33
Lab equipment	296	159
	1,505	667
Less: Accumulated depreciation and amortization	(171)	(264)
	$ 1,334	$ 403

Depreciation and amortization expense related to property and equipment was $248 thousand and $71 thousand for the nine months ended September 30, 2020 and 2019 respectively.

6. Accrued Expenses

Accrued expenses consisted of the following:

(in thousands)	September 30, 2020	December 31, 2019
Accrued employee compensation and benefits	$ 3,968	2,916
Accrued external research and development expenses	3,086	1,977
Accrued professional fees	768	1,347
Accrued lease construction costs	207	—
Other	503	221
	$ 8,532	$ 6,461

7. Long-Term Debt

Long-term debt consisted of the following:

(in thousands)	September 30, 2020	December 31, 2019
Principal amount of long-term debt	$ 25,000	$ 20,000
Debt discount, net of accretion	229	(317)
Cumulative accretion of final payment due at maturity	308	414
Long-term debt, including accretion	$ 25,537	$ 20,097

Hercules Loan Agreements

In October 2018, the Company entered into the Loan and Security Agreement, as amended in December 2019 (the "Hercules Loan Agreement"), under which the Company borrowed an aggregate of $10.0 million under a term loan. In June 2019, the Company refinanced the Hercules Loan Agreement and entered into an Amended and Restated Loan and Security Agreement (the "Amended and Restated Loan Agreement") with Hercules. The Amended and Restated Loan Agreement provided for aggregate maximum borrowings of $35.0 million, under which $20.0 million was borrowed, including $10.0 million that was previously outstanding and $10.0 million in new borrowings. Borrowings under the Amended and Restated Agreement accrued interest at a variable rate equal to the greater of (i) 8.75% or (ii) *The Wall Street Journal* prime rate plus 2.75%. In an event of default and until such event is no longer continuing, the interest rate applicable to borrowings under the Amended and Restated Agreement would be increased by 4.0%.

On March 13, 2020, the Company entered into a First Amendment to the Amended and Restated Loan and Security Agreement dated June 27, 2019 (collectively the "Amended Loan Agreement") with Hercules, which provides for aggregate maximum borrowings of up to $50.0 million. The Amended Loan Agreement provides for (i) a term loan of $25.0 million, including the $20.0 million previously outstanding under the Amended and Restated Loan Agreement and an additional $5.0 million drawn at the closing of the first amendment on March 13, 2020, (ii) subject to the achievement of certain performance milestones and other conditions, a right of the Company to request that Hercules make additional term loan advances in an aggregate amount of up to $7.5 million through June 30, 2021, (iii) subject to the achievement of certain performance milestones and conditions, a right of the Company to request that Hercules make additional term loan advances in an aggregate amount of up to $7.5 million through June 30, 2022 and (iv) subject to Hercules investment committee's sole discretion, a right of the Company to request that Hercules make additional term loan advances in an aggregate amount of up to $10.0 million through December 31, 2022.

Borrowings under the Amended Loan Agreement bear interest at a variable rate equal to a per annum rate of interest equal to the greater of either (i) 3.75% plus the prime rate as reported in *The Wall Street Journal*, and (ii) 8.75%. In an event of default, as defined in the Amended Loan Agreement, and until such event is no longer continuing, the interest rate applicable to borrowings under the Amended Loan Agreement would be increased by 4.0%.

Borrowings under the Amended Loan Agreement are repayable in monthly interest-only payments through January 1, 2022, and in equal monthly payments of principal and accrued interest from February 1, 2022 until the maturity date of the loan, which is July 1, 2023. The Company may prepay all, but not less than all, of the outstanding borrowings, subject to a

prepayment premium of up to 2.0%, 1.0% or 0.5% of the principal amount outstanding as of the date of repayment, in each case depending on when such repayment is made. In addition, the Amended Loan Agreement provides for payments of (i) $0.8 million payable upon the earlier of November 1, 2021 or the repayment in full of all obligations under the Amended Loan Agreement, and (ii) 4.0% of the aggregate principal amount of all Term Loan Advances drawn under the Amended Loan Agreement, payable upon the earlier of the maturity of the Amended Loan Agreement or the repayment in full of all obligations under the Amended Loan Agreement.

Borrowings under the Amended Loan Agreement are collateralized by substantially all of the Company's personal property and other assets except for their intellectual property (but including rights to payment and proceeds from the sale, licensing or disposition of the intellectual property). Under the Amended Loan Agreement, the Company has agreed to affirmative and negative covenants to which it will remain subject until maturity or repayment of the loan in full. The covenants include, without limitation:

(a) Effective upon the date the outstanding principal amount of the advances under the Amended Loan Agreement exceeds $25.0 million, which has not yet occurred, the Company at all times thereafter must maintain cash in an account or accounts in which Hercules has a first priority security interest, in an aggregate amount greater than or equal to the greater of (i) $30.0 million or (ii) 6 multiplied by a metric based on prior months' cash expenditures ("RML"); provided, however, that from and after the Company's achievement of certain performance milestones, the required level shall be reduced to the greater of (x) $20.0 million, or (y) 3 multiplied by the current RML; and provided further, that subject to the achievement of certain milestones, this covenant shall be extinguished.

(b) Restrictions on 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's obligations under the Amended Loan Agreement are subject to acceleration upon the occurrence of specified events of default, including payment default, insolvency and a material adverse change in the Borrower's business, operations or financial or other condition. In addition, under the Amended Loan Agreement, Hercules has the right to participate, in a cumulative amount of up to $3.0 million in the aggregate, of which $1.0 million has already been exercised, and subject to exceptions as provided in the Amended Loan Agreement, in any future offering of the Company's equity securities for cash that is solely for financing purposes and is broadly marketed to multiple investors.

The Company concluded that the previous debt under the Amended and Restated Loan Agreement was extinguished based on the difference in the cash flows of the previous and new debt. Accordingly, the difference between the carrying amount of the previous debt, including the unamortized debt discount, and the fair value of the new debt under the Amended Loan Agreement was recorded as a $162 thousand loss on extinguishment of debt for the nine months ended September 30, 2020. Legal and consulting fees paid to third parties directly related to the new debt have been deferred and will be amortized to interest expense over the life of the new debt arrangement using the effective interest method.

The Company recognized aggregate interest expense under its loan agreements with Hercules of $0.7 million and $2.0 million during the three and nine months ended September 30, 2020, respectively, and $0.4 million and $0.7 million for the three and nine months ended September 30, 2019, respectively. Interest expense includes $0.1 million and $0.4 million for the three and nine months ended September 30, 2020, respectively, related to the accretion of the debt discount and the final payment. The annual effective interest rate of the Amended Loan Agreement as of September 30, 2020 is 10.7%. There were no principal payments due or paid under the Amended Loan Agreement during the nine months ended September 30, 2020.

As of September 30, 2020, future principal payments and the final payment due under the Amended Loan Agreement were as follows (in thousands):

Year Ending December 31,	Total
2020	$ —
2021	—
2022	14,871
2023	10,129
Long-term debt	$ 25,000

8. Leases

The Company has lease agreements for its facilities in Boston, Massachusetts, which is the Company's principal executive office; Vienna, Austria, which is the Company's research and development center; and Waltham, Massachusetts, Arsanis' former research offices which the Company has sublet to a third party. The Company terminated its former office lease in Cambridge, Massachusetts as of September 30, 2020 as described below. There are no restrictions or financial covenants associated with any of the lease agreements.

Cambridge Lease— In August 2017, the Company entered into a non-cancellable operating lease agreement for office space of approximately thirteen thousand square feet in Cambridge, Massachusetts ("Cambridge Lease") which had an original expiration date of July 31, 2022. The Company reached an agreement with the landlord on July 17, 2020 to terminate the lease effective September 30, 2020 in exchange for a fee. The Company accounted for the lease modification prospectively from the date of the termination through the new lease termination date of September 30, 2020. As of September 30, 2020, the right-of-use asset and associated lease liabilities were retired from the condensed consolidated balance sheet. The Company must maintain a letter of credit of $264 thousand, which is secured by restricted cash, for the benefit of the landlord until October 30, 2020.

Waltham Lease— The Company leases approximately six thousand square feet of office space in Waltham, Massachusetts ("Waltham Lease"). The Waltham Lease, as amended, commenced on January 1, 2019, and expires approximately five years from the commencement date. The base rent is approximately $262 thousand 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.

Vienna Austria Leases— The Company has an operating lease, as amended, for approximately 400 square meters of laboratory and office space in Vienna, Austria ("2019 Vienna Lease"), which commenced on March 1, 2019, as amended, for a term of 2 years and is cancellable by the Company upon three months' notice with no penalty. The annual base rent for the 2019 Vienna Lease is approximately $155 thousand. The Company classified the 2019 Vienna Lease as a short-term lease as it is not reasonably certain that the Company would not terminate the lease within one year and, accordingly, the lease is not recorded as a right-of-use asset. In September 2020, the Company entered into a new operating lease for approximately 1,200 square meters of laboratory and office space in Vienna, Austria ("Vienna Lease"), which is expected to commence in March 2021 for a term of 7 years and will replace the 2019 Vienna Lease. The Company will contribute approximately $575 thousand to building improvements in connection with the Vienna Lease. The annual base rent for the Vienna Lease, following a 6-month rent free period, will be approximately $300 thousand. The Company will record a right-of-use asset and associated lease liabilities upon the commencement of the Vienna Lease in 2021.

Boston Lease— On November 11, 2019, the Company entered into a lease agreement for approximately 28,000 square feet of office space that was subsequently constructed in a vacant building located in Boston, Massachusetts ("Boston Lease"). The office space is the Company's current headquarters replacing the Cambridge Lease. Monthly rent payments under the Boston Lease commenced in May 2020. Base rental payments will be approximately $1.0 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 $1.1 million for the benefit of the landlord.

For the Boston lease, the Company participated in the construction of the office space and has incurred construction costs to prepare the office space for its use, which have been partially reimbursed by the landlord. The Company has concluded that these construction costs generate and enhance the landlord's assets, and, as such, unreimbursed construction costs incurred by the Company to prepare the office space for its use have been classified a part of the right-of-use asset. The commencement date for the Boston Lease was September 1, 2020 upon substantial completion of the landlord's asset. On the commencement date, the Company recognized a lease liability of $4.6 million reflecting the future rent payments for the term of the Boston

Lease discounted at the Company's collateralized borrowing rate, and a right-of-use asset of $8.0 million measured as the lease liability plus rent payments made and unreimbursed construction costs incurred prior to the commencement date. The Company began recognizing rent expense following the commencement date.

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, 2020 and 2019 were as follows (dollars in thousands):

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
Lease Cost	**2020**	**2019**	**2020**	**2019**
Fixed operating lease cost	$ 413	$ 225	$ 847	$ 610
Short-term lease costs	40	38	116	84
Total lease expense	$ 453	$ 263	$ 963	$ 694
Other information				
Right-of-use asset obtained in exchange for operating lease liabilities	$ 4,646			
Operating cash flows from operating leases	$ 479	$ 268	$ 963	$ 737
Sublease income	$ 49	$ —	$ 146	$ —
Weighted-average remaining lease term—operating leases	5.8 years			
Weighted-average discount rate—operating leases	11.2 %			

Maturities of lease liabilities due under lease agreements that have commenced as of September 30, 2020 are as follows (in thousands):

Maturity of lease liabilities	**Operating Leases**
2020	$ 313
2021	1,273
2022	1,301
2023	1,329
2024	1,094
2025	1,122
Thereafter	1,052
Total lease payments	7,484
Less: interest	(2,051)
Total operating lease liabilities as of September 30, 2020	$ 5,433

9. Commitments and Contingencies

The Company has agreements with clinical research organizations ("CROs") pursuant to which the Company and the CROs are conducting clinical trials of mavorixafor for the treatment of WHIM syndrome, Waldenström's and SCN. The Company may terminate these agreements by providing notice pursuant to the contractual provisions of such agreements and would incur early termination fees. The Company also has agreements with contract manufacturing organizations ("CMOs") for the production of mavorixafor for use in clinical trials.

***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, 2020 or December 31, 2019.

***License Agreements*—** In February 2017, Arsanis entered into an option and license agreement with Adimab to obtain rights to certain RSV antibodies, which are being used in the ASN500 program. The Company exercised this option for an up-front payment of $250 thousand. In July 2019, the Company transferred the intellectual property related to the ASN500 program through an exclusive, worldwide license with a third party. The third party subsequently cancelled the sublicense agreement effective October 2020. The Company is obligated to make payments to Adimab based on future clinical and regulatory milestones of up to approximately $25.0 million, as well as royalty payments on a product-by-product and country-by-country basis of a mid-single-digit percentage based on net sales of products based on certain RSV antibodies during the applicable term for such product in that country.

***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. Preferred and Common Stock Warrants

In connection with its issuance of common stock in public offerings that closed on April 16, 2019 and November 29, 2019, the Company issued 3,900,000 Class A warrants, which are exercisable for the Company’s common stock, and 5,416,667 Class B warrants, which are exercisable for shares of the Company’s common stock or prefunded warrants to purchase shares of the Company's common stock. The Class A warrants have an exercise price of $13.20 per share, expire on April 15, 2024 and were immediately exercisable. The Class B warrants were immediately exercisable upon issuance, have an initial exercise price of $15.00 per share and expire on a date that is the earlier of (a) the date that is 30 calendar days from the date on which the Company issues a press release announcing top-line data from its Phase 3 clinical trial of mavorixafor for the treatment of patients with WHIM syndrome (or, if such date is not a business day, the next business day) and (b) November 28, 2024. The Class B warrants have a contingent price adjustment feature pursuant to which the exercise price of the Class B warrants will be adjusted to the lowest weighted average offering price at which the Company sells its common stock or certain securities convertible into or exercisable for the Company's common stock in one or more subsequent offerings, if the weighted average offering price for such offering is below $15.00. As of September 30, 2020, no such subsequent offerings have occurred but may in the future. In addition, in connection with the April 16, 2019 and November 29, 2019 offerings, the Company issued 2,130,000 and 1,750,000 prefunded warrants, respectively, for proceeds of $10.999 and $11.999 per share, respectively. Each of the prefunded warrants is exercisable into one share of the Company's common stock, has a remaining exercise price of $0.001 per share and was immediately exercisable upon issuance.

Prior to the Merger (see Note 1), the Company issued warrants for the purchase of its convertible preferred stock. Upon the closing of the Merger, pursuant to the Merger Agreement, all of the outstanding X4 preferred stock was converted to Arsanis common stock and the X4 preferred stock warrants converted to warrants for the purchase of common stock.

The following table provides a roll forward of outstanding warrants for the nine month period ended September 30, 2019:

	Number of warrants	Weighted Average Exercise Price	Weighted Average Contractual Term (Years)
Outstanding and exercisable warrants to purchase preferred shares as of December 31, 2018	5,146,400		4.23
Conversion of warrants to purchase preferred shares to warrants for the purchase of common stock and adjusted for the Exchange Ratio and Reverse Stock Split	(4,657,350)		
Issuance of warrants for the purchase of common stock	6,035,000	12.43	
Exercised	(33,846)	13.20	
Outstanding warrants to purchase common shares as of September 30, 2019	6,490,204	$ 13.03	2.98

The following table provides a roll forward of outstanding warrants for the nine month period ended September 30, 2020:

	Number of warrants	Weighted Average Exercise Price	Weighted Average Contractual Term (Years)
Outstanding and exercisable warrants to purchase common shares as of December 31, 2019	13,656,871	$13.68	4.59
Cancelled	(150,831)		
Outstanding and exercisable warrants to purchase common shares as of September 30, 2020	13,506,040	$13.59	3.90

As of September 30, 2020, the Company's outstanding warrants to purchase shares of common stock consisted of the following:

Issuance Date	Number of Shares of Common Stock Issuable	Exercise Price	Classification	Expiration Date
October 25, 2016	5,155	$ 19.78	Equity	October 24, 2026
November 1, 2017	130,609	$ 19.78	Equity	October 31, 2020
November 17, 2017	8,442	$ 19.78	Equity	November 16, 2020
December 4, 2017	5,661	$ 19.78	Equity	December 3, 2020
December 28, 2017	6,925	$ 19.78	Equity	December 27, 2020
December 28, 2017	115,916	$ 19.78	Equity	December 28, 2027
September 12, 2018	25,275	$ 19.78	Equity	September 12, 2021
September 12, 2018	20,220	$ 19.78	Equity	September 12, 2028
October 19, 2018	20,016	$ 19.78	Equity	October 19, 2028
March 13, 2019	5,000	$ 19.80	Equity	March 12, 2029
April 16, 2019	3,866,154	$ 13.20	Equity	April 15, 2024
April 16, 2019	2,130,000	$ 11.00	Equity	n/a
November 29, 2019	5,416,667	$ 15.00	Equity	November 28, 2024
November 29, 2019	1,750,000	$ 12.00	Equity	n/a
	13,506,040			

11. Common Stock

Common Stock— On June 10, 2020, the Company's stockholders approved an amendment to the Company's Restated Certificate of Incorporation, as amended, to increase the number of the Company's authorized shares of common stock, par value $0.001 per shares, from 33,333,333 shares to 125,000,000 shares. As of September 30, 2020, the Company's Restated Certificate of Incorporation, as amended through such date (the "Restated Certificate"), authorized the Company to issue 125,000,000 shares of common stock. The voting, dividend and liquidation rights of the holders of the Company's common stock are subject to and qualified by the rights, powers and preferences of the holders of 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.

On August 7, 2020, the Company entered into a Controlled Equity Offering^{SM} Sales Agreement (the "ATM Sales Agreement") with B. Riley Securities, Inc., Cantor Fitzgerald & Co., and Stifel, Nicolaus & Company, Incorporated (collectively the "Sales Agents"), pursuant to which the Company may offer and sell, at the Company's sole discretion through one or more of the Sales Agents, shares of its common stock having an aggregate offering price of up to $50.0 million. The Sales Agents may sell the common stock by any method deemed to be an "at the market offering" as defined in Rule 415(a)(4) under the Securities Act of 1933, as amended, including, without limitation, sales made directly on the Nasdaq Capital Market or any other existing trading market for the Company's common stock. Any shares of common stock sold will be issued pursuant to the Company's shelf registration statement on Form S-3 (File No. 33-242372). No shares of common stock were sold pursuant to the ATM Sales Agreement during the three or nine months ended September 30, 2020.

On October 14, 2020, the Company entered into a common stock purchase agreement (the "Aspire Purchase Agreement") with Aspire Capital Fund, LLC, ("Aspire Capital"), which provides that, upon the terms and subject to the conditions and limitations set forth in the Aspire Purchase Agreement, Aspire Capital is committed to purchase up to an aggregate of $50.0 million of shares of the Company's common stock at the Company's request from time to time during the 36-month term of the Purchase Agreement. Concurrently with entry into the Aspire Purchase Agreement, the Company also entered into a registration rights agreement with Aspire Capital (the "Registration Rights Agreement"), pursuant to which the Company filed with the SEC a prospectus supplement to the Company's shelf registration statement on Form S-3 (File No. 333-242372), registering the

issuance and sale of common stock that the Company may offer to Aspire Capital from time to time under the Aspire Purchase Agreement.

12. Stock-Based Compensation

***Summary of Plans*—** Upon completion of the Merger with Arsanis on March 13, 2019, X4's 2015 Employee, Director and Consultant Equity Incentive Plan, as amended (the "2015 Plan"), Arsanis' 2017 Equity Incentive Plan (the "2017 Plan") and Arsanis' 2017 Employee Stock Purchase Plan (the "2017 ESPP") were assumed by the Company. In June 2019, the Company adopted the 2019 Inducement Equity Incentive Plan (the "Inducement Plan" and collectively with the 2015 Plan, 2017 Plan and 2017 ESPP, the "Plans"). 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.

***2015 Employee, Director and Consultant Equity Incentive Plan*—** In 2015, the Board of Directors and shareholders of the Company adopted the 2015 Plan, which provides for the Company to grant incentive stock options or nonqualified stock options, restricted stock awards and other stock-based awards to employees, directors and consultants of the Company. The total number of shares of common stock that may be issued under the 2015 Plan is 969,340 shares. Shares that are expired, forfeited, canceled or otherwise terminated without having been fully exercised will be available for future grant under the 2015 Plan. As of November 2, 2020, there were a *de minimis* number shares available for issuance under the 2015 Plan.

***2017 Equity Incentive Plan*—** Under the 2017 Plan, the Company may grant incentive stock options, non-qualified options, stock appreciation rights, restricted stock awards, restricted stock units and other stock-based awards. Incentive stock options may be granted only to the Company's employees, including officers and directors who are also employees. Awards other than incentive stock options may be granted to employees, officers, members of the board of directors, advisors and consultants of the Company. On June 10, 2020, the Company's stockholders approved the amendment and restatement of the 2017 Plan (the "Amended and Restated 2017 Plan"). The amendments increased the number of shares of common stock reserved for issuance under the 2017 Plan by an additional 474,465 shares (subject to adjustment for certain corporate events) and amended the "evergreen" provision of the 2017 Plan to provide for an automatic increase in the share reserve on the first day of each year, beginning on January 1, 2021 and ending on January 1, 2027, by an amount equal to the lesser of (a) 4% of the Company's outstanding shares on such date and (b) a number of shares determined by the Board of Directors. As of November 2, 2020, approximately 30,000 shares were available for future issuance under the Amended and Restated 2017 Plan.

***2017 Employee Stock Purchase Plan*—** The 2017 ESPP provides participating employees with the opportunity to purchase shares of the Company's common stock at defined purchase prices over six-month offering periods. For the nine months ended September 30, 2020, 10,057 shares of common stock were issued under the 2017 ESPP.

***2019 Inducement Equity Incentive Plan*—** On June 17, 2019, the Board of Directors approved the adoption of the Inducement Plan, which is used exclusively for the grant of equity awards to individuals who were not previously employees of the Company (or following a bona fide period of non-employment), as an inducement material to such individual's entering into employment with the Company, pursuant to Nasdaq Listing Rule 5635(c)(4). The Board of Directors approved amendments to the Inducement Plan in December 2019 and October 2020, increasing the total number of shares of common stock that may be issued under the Inducement Plan to 650,000 shares. Shares that are expired, forfeited, canceled or otherwise terminated without having been fully exercised will be available for future grant under the Inducement Plan. In addition, shares of common stock that are tendered to the Company by a participant to exercise an award are added to the number of shares of common stock available for future grants. As of November 2, 2020, approximately 290,000 shares were available for future issuance under the Inducement Plan.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Risk-free interest rate	0.4 %	1.6 %	0.6 %	2.0 %
Expected term (in years)	6.1	6.0	6.0	6.0
Expected volatility	95.1 %	84.5 %	94.9 %	88.0 %
Expected dividend yield	0 %	0 %	0 %	0 %

Stock Options

The following table summarizes the Company's stock option activity for the nine months ended September 30, 2020:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Contractual Term (Years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2019	1,297,029	$ 17.05	8.4	$ 1,286
Granted	498,662	9.13		
Exercised	(17,689)	7.25		
Forfeited	(164,978)	21.68		
Outstanding as of September 30, 2020	1,613,024	$ 14.24	8.2	$ —
Exercisable as of September 30, 2020	682,632	$ 18.23	7.0	$ —
Vested and expected to vest as of September 30, 2020	1,404,246	$ 14.43	8.1	$ —

The aggregate intrinsic value of options is calculated as the difference between the exercise price of the stock options and the fair value of the Company's common stock for those options that had exercise prices lower than the fair value of the Company's common stock. The aggregate intrinsic value of stock options exercised during the nine months ended September 30, 2020 and 2019 was $43 thousand and $184 thousand, respectively. The weighted average grant-date fair value per share of stock options granted during the nine months ended September 30, 2020 and 2019 was $6.93 and $11.33, respectively.

Restricted Stock Units— The following table summarizes the Company's restricted stock unit activity for the nine months ended September 30, 2020

	Number of Shares
Unvested as of December 31, 2019	101,773
Granted	681,081
Vested	(131,574)
Forfeited	(39,291)
Unvested as of September 30, 2020	611,989

During the nine months ended September 30, 2020, the Company granted 678,581 performance based restricted stock units, which vest in part based on the Company's achievement of operational milestones and over time thereafter for the subsequent two years as the grantee continues to provide services to the Company. As of September 30, 2020, two out of the four performance criteria had been met. The Company believes that the achievement of the remaining operational milestones is

probable and, accordingly, stock-based compensation expense has been recognized for the awards using the accelerated attribution model based on the fair value of the awards as of the date of grant and management's best estimate of the date each operational milestone will be achieved. The Company will update 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, 2020, total unrecognized compensation expense related to unvested stock options and restricted stock units was $9.1 million, which is expected to be recognized over a weighted average period of 2.3 years.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Research and development expense	$ 1,006	$ 301	$ 1,711	$ 599
General and administrative expense	1,238	390	2,319	787
Total stock-based compensation	$ 2,244	$ 691	4,030	1,386

13. Income Taxes

The Company did not record a federal or state income tax benefit for its losses for the three months ended September 30, 2020 and 2019, respectively, or the nine month period ended September 30, 2019, due to the conclusion that a full valuation allowance is required against the Company's deferred tax assets in all jurisdictions. For the nine months ended September 30, 2020, the Company recorded a tax provision of $148 thousand related to local withholdings on a milestone payment received from a customer in a foreign jurisdiction.

14. Net Loss per Share

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in thousands, except per share data)	2020	2019	2020	2019
Numerator:				
Net loss	$ (17,449)	$ (17,714)	$ (43,731)	$ (41,970)
Accruing dividends on Series A convertible preferred stock	—	—	—	(592)
Net loss attributable to common stockholders	$ (17,449)	$ (17,714)	$ (43,731)	$ (42,562)
Denominator:				
Weighted average common shares outstanding—basic and diluted	20,085	14,562	20,035	9,866
Net loss per share attributable to common stockholders— basic and diluted	$ (0.87)	$ (1.22)	$ (2.18)	$ (4.31)

The Company has included 107,371 shares of redeemable common stock, which were converted to common shares upon the Merger on March 13, 2019, in its computation of basic and diluted weighted average common shares outstanding for the nine months ended September 30, 2019 as this class of stock participated in losses similarly to other common stockholders. Basic and diluted weighted average common shares outstanding for the three and nine months ended September 30, 2020 also include the weighted average effect of 3,880,000 pre-funded warrants for the purchase of common shares for which the remaining unfunded exercise price is less than $0.01 per share.

The Company's potentially dilutive securities include outstanding stock options, restricted stock units and warrants to purchase shares of common stock for the three and nine months ended September 30, 2020 and 2019. 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 common shares outstanding used to calculate both basic and diluted net loss per share attributable to common stockholders is the same. The Company excluded the following potential common shares, 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,	
	2020	2019	2020	2019
Options to purchase common stock	1,613,024	1,271,141	1,613,024	1,271,141
Unvested restricted stock units	611,994	116,689	611,994	116,689
Warrants to purchase common stock (excluding prefunded warrants, which are included in basic shares outstanding)	9,626,040	4,360,204	9,626,040	4,360,204
	11,851,058	5,748,034	11,851,058	5,748,034

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, or the SEC, on March 12, 2020, or 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. Such factors may be amplified by the ongoing COVID-19 pandemic and its potential impact on our business and the overall global economy.

Overview

We are a late-stage clinical biopharmaceutical company focused on the research, development and commercialization of novel therapeutics for the treatment of rare diseases. Our pipeline is comprised of potentially first-in-class, oral, small molecule antagonists of chemokine receptor CXCR4, which have the potential to treat a broad range of rare diseases, including primary immunodeficiencies, or PIs, and certain types of cancer. PIs are a group of more than 250 rare, chronic disorders in which flaws in the immune system cause increased susceptibility to infections and, in some cases, increased risk of cancers. Within this broad disease classification, a number of PIs are attributed to the improper trafficking of immune cells related to the CXCR4 receptor and its ligand CXCL12. CXCR4 is stimulated by its only chemokine ligand, CXCL12, and plays a key role in enabling the trafficking of immune cells and effectively monitoring the function of the immune system, or immunosurveillance. Overstimulation of the CXCL12/CXCR4 pathway leads to inhibition of the immune response, or immunosuppression. Our lead product candidate, mavorixafor, is a potentially first-in-class, oral, allosteric antagonist of the CXCR4 receptor designed to correct the abnormal signaling caused by the receptor/ligand interaction and enable mobilization and trafficking of immune cells.

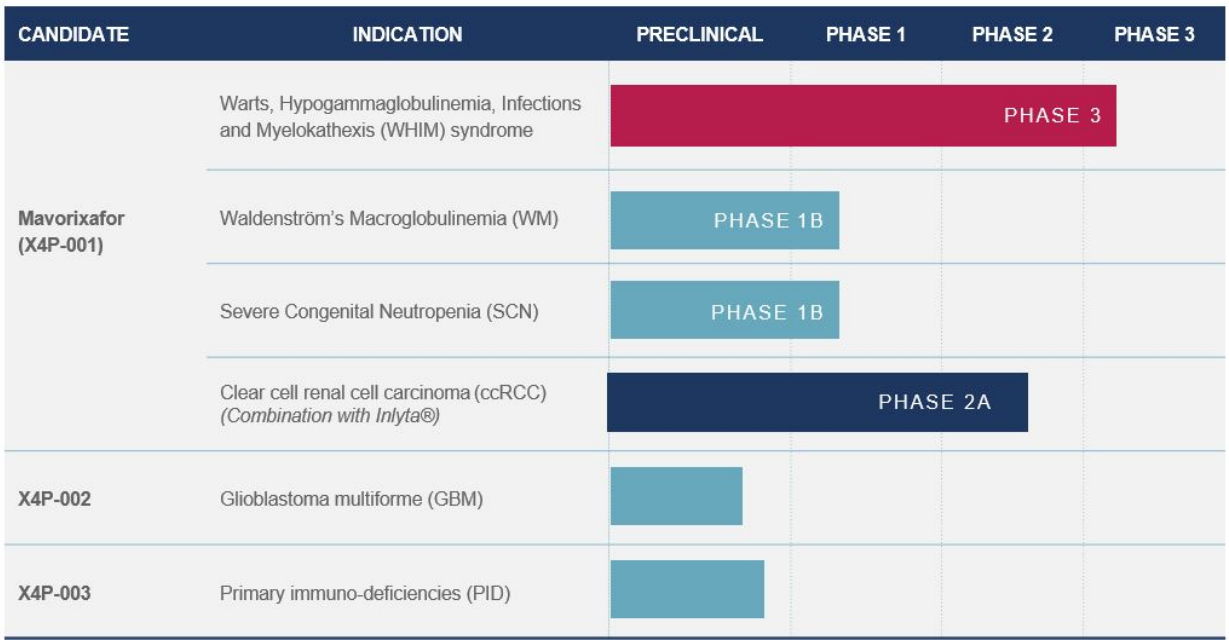
We have completed a Phase 2 clinical trial of mavorixafor in patients with Warts, Hypogammaglobulinemia, Infections, and Myelokathexis, or WHIM, syndrome, which is a rare, inherited primary immunodeficiency disease. In June 2019, we initiated 4WHIM, a pivotal, 52-week Phase 3 global clinical trial of mavorixafor for the treatment of patients with WHIM syndrome. In November 2019, the U.S. Food and Drug Administration, or FDA, granted Breakthrough Therapy Designation for mavorixafor for the treatment of adults with WHIM and in October 2020, the FDA granted Fast Track Designation for mavorixafor for adults with WHIM. We continue to expect that the timing of reporting of top-line data from this trial will be delayed into 2022 due to the impact of the ongoing COVID pandemic. We are also investigating mavorixafor in combination with axitinib (Inlyta®), an FDA-approved small molecule tyrosine kinase inhibitor, in the Phase 2a portion of an open-label Phase 1/2 clinical trial in patients with clear cell renal cell carcinoma, or ccRCC. In September 2019, we announced positive results from the Phase 2a portion of this trial. We plan to pursue future development and potential commercialization of mavorixafor in ccRCC and other possible immuno-oncology indications outside of greater China only as part of a potential strategic collaboration.

In November 2019, we initiated a 14-day, proof-of-concept Phase 1b clinical trial of mavorixafor in patients with severe congenital neutropenia, or SCN. We currently expect to report initial data from the SCN trial in 2021. In December 2019, we initiated a Phase 1b clinical trial of mavorixafor, in combination with ibrutinib, in patients with Waldenström's macroglobulinemia, or Waldenström's. The reporting of initial data from the Waldenström's trial will be delayed into the first half of 2021 due to delays in clinical site activation and the slower than expected pace of patient enrollment caused by the ongoing COVID-19 pandemic.

We are also advancing two early stage candidates towards the clinic: X4P-003, a second-generation CXCR4 antagonist designed to have an enhanced pharmacokinetic profile relative to mavorixafor, potentially enabling improved patient compliance and ease of use; and X4P-002, a CXCR4 antagonist designed to cross blood-brain barrier and provide appropriate therapeutic exposures to treat brain cancers.

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.

Our Pipeline



COVID-19 Business Update

In light of the ongoing COVID-19 pandemic, we have implemented business continuity measures designed to address and mitigate the impact of the COVID-19 pandemic on our employees, our business, including our clinical trials, supply chains and third-party providers. We continue to closely monitor the COVID-19 pandemic as we evolve our business continuity plans and response strategy. In March 2020, the Governor of Massachusetts ordered all individuals living in the Commonwealth of Massachusetts to stay at their place of residence for an indefinite period of time (subject to certain exceptions to facilitate authorized necessary activities) to mitigate the impact of the COVID-19 pandemic. As a result, our workforce transitioned to working remotely and we continued to work remotely through September 30, 2020, even as governmental restrictions have started to relax. In the fourth quarter of 2020, we opened our new office in Boston, Massachusetts under a return-to-work plan with a limited phased approach that is principles-based, local in design, with focused on employee safety and optimal work environment. While we are experiencing limited financial impacts at this time, given the global economic slowdown, the overall disruption of global healthcare systems and the other risks and uncertainties associated with the pandemic, our business, financial condition, results of operations and growth prospects could be materially adversely affected.

Clinical Development

With respect to clinical development, we continue to implement risk-based approaches in accordance with FDA and the European Medicines Agency ("EMA") COVID-19 guidance, which includes virtual and remote patient visits and monitoring where possible, while prioritizing patient safety, maintaining trial continuity and preserving data integrity. We have experienced, and expect to continue to experience, a disruption or delay in our ability to initiate trial sites and/or enroll and assess patients in several of our clinical programs as a result of the ongoing COVID-19 pandemic. We have experienced challenges in providing trial drugs to patients enrolled in our clinical trials, and where necessary and practical, have implemented direct-to-patient shipments from clinical sites. While not currently impacted, we could foresee an impact on our ability to supply study drug, report trial results, or interact with regulators, ethics committees or other important agencies due to limitations in regulatory authority employee resources or otherwise. In addition, we rely on contract research organizations, or CROs, or other third parties to assist us with clinical trials, and we cannot guarantee that they will continue to perform their contractual duties in a timely and satisfactory manner as a result of the COVID-19 pandemic. If the COVID-19 pandemic continues and persists for an extended period of time, we could experience further disruptions to our clinical development timelines, which would adversely affect our business, financial condition, results of operations and growth prospects.

Supply Chain

We continue to work closely with our third-party manufacturers, distributors and other partners to manage our supply chain activities and mitigate potential disruptions to our clinical supply as a result of the COVID-19 pandemic. We have business continuity plans in place and expect to have adequate global supply of mavorixafor through 2020 and well into 2021. To best

support our patients, we are working with our vendors to provide the option for direct-to-patient drug shipments from clinical sites. If the COVID-19 pandemic begins to impact essential distribution systems we could experience disruptions to our supply chain and operations, which would adversely impact our ability to carry out our clinical trials.

Regulatory Activities
We expect that we could experience delays in the timing of review and/or our interactions with the FDA or the European Commission, or EC, due to, for example, absenteeism by governmental employees, inability to conduct planned physical inspections related to regulatory approval, or the diversion of efforts of the FDA or EC and attention to approval of other therapeutics or other activities related to COVID-19, which could delay approval decisions with respect to the preparation and submission to the FDA of a new drug application, or NDA, or the preparation and submission to the EC of a Marketing Authorization Application, or MAA, and otherwise delay or limit our ability to make planned regulatory submissions or obtain new product approvals.

Financial Impact
The COVID-19 pandemic continues to rapidly evolve and has already resulted in a significant disruption of global financial markets. If the disruption persists and deepens, we could experience an inability to access additional capital, which could in the future negatively affect our operations.

Results of Operations

Comparison of the Three and Nine Months Ended September 30, 2020 and 2019

The following table summarizes the results of our operations for the three and nine months ended September 30, 2020 and 2019 (in thousands):

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2020	2019	Change	2020	2019	Change
	(in thousands)			(in thousands)		
License revenue	$ —	$ —	$ —	$ 3,000	$ —	$ 3,000
Operating expenses:						
Research and development	11,381	8,589	2,792	29,634	23,098	6,536
General and administrative	5,599	4,383	1,216	15,585	13,726	1,859
Loss on transfer of non-financial assets	—	4,004	(4,004)	—	4,004	(4,004)
Total operating expenses	16,980	16,976	4	45,219	40,828	4,391
Loss from operations	(16,980)	(16,976)	(4)	(42,219)	(40,828)	(1,391)
Other expense, net:						
Interest income	—	464	(464)	272	927	(655)
Interest expense	(697)	(688)	(9)	(1,968)	(1,599)	(369)
Change in fair value of preferred stock warrant and derivative liabilities	—	—	—	—	(105)	105
Loss on extinguishment of debt	—	(566)	566	(162)	(566)	404
Other income	228	52	176	494	201	293
Total other expense, net	(469)	(738)	269	(1,364)	(1,142)	(222)
Loss before provision for income taxes	(17,449)	(17,714)	265	(43,583)	(41,970)	(1,613)
Provision for income taxes	—	—	—	148	—	148
Net loss	$ (17,449)	$ (17,714)	$ 265	$ (43,731)	$ (41,970)	$ (1,761)

License Revenue
There was no license revenue recorded for the three months ended September 30, 2020 or 2019. For the nine months ended September 30, 2020, we recorded $3.0 million in revenue related to our license agreement with Abbisko Therapeutics Co., Ltd., or Abbisko, due to the achievement of a financial milestone in March 2020. There was no similar revenue recorded in the nine months ended September 30, 2019. Pursuant to our license agreement with Abbisko, we are eligible to receive potential development and regulatory milestone payments, which vary based on the number of indications developed, and potential commercial milestone payments based on annual net sales of mavorixafor-based licensed products. The transaction price related to these milestones has been allocated to the delivery to Abbisko of the license to develop and commercialize mavorixafor, was satisfied at the inception of the arrangement in July 2019 and has been fully constrained. We have determined that the future sales of clinical and commercial supplies to Abbisko represent optional goods that will be recognized as revenue at a point in time when such clinical or commercial supply is delivered to Abbisko in the future. With respect to the remaining operational

milestones, we will re-evaluate the constraint on the transaction price associated with these milestones in each reporting period and as uncertain events are resolved or other changes in circumstances occur.

Research and Development Expenses

Research and development expenses consist primarily of costs incurred in connection with the discovery and development of our product candidates, including employee salaries and related expenses, expenses incurred in connection with the preclinical and clinical development of our product candidates, including under agreements with third parties, such as consultants and CROs; the cost of manufacturing drug products for use in our preclinical studies and clinical trials, including under agreements with third parties, such as consultants and contract manufacturing organizations, or CMOs; facilities, depreciation and other expenses, which include direct or allocated expenses for rent and maintenance of facilities and insurance; costs related to compliance with regulatory requirements; and payments made under third-party licensing agreements. We expense research and development costs as incurred.

Research and Development Expenses

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2020	2019	Change	2020	2019	Change
	(in thousands)			(in thousands)		
Direct research and development expenses by product candidate:						
Mavorixafor (X4P-001)	$ 6,173	$ 5,137	$ 1,036	$ 15,792	$ 13,407	$ 2,385
X4P-002	282	78	204	790	219	571
X4P-003	630	276	354	962	494	468
Unallocated expense	4,296	3,098	1,198	12,090	8,978	3,112
Total research and development expenses	$ 11,381	$ 8,589	$ 2,792	$ 29,634	$ 23,098	$ 6,536

Research and development expenses were $11.4 million for the three months ended September 30, 2020, compared to $8.6 million for the three months ended September 30, 2019. Research and development expenses were $29.6 million for the nine months ended September 30, 2020, compared to $23.1 million for the nine months ended September 30, 2019. The increase in research and development expenses for the three and nine months ended September 30, 2020 as compared to the same period in the prior year was primarily due to higher clinical expenses related to mavorixafor to support our three ongoing clinical trials, an increase in outsourced costs related to our early stage drug candidates and an increase in unallocated expenses, primarily due to an increase in compensation expenses, including stock-based compensation, due to additional personnel within our manufacturing, regulatory and clinical operations functions.

General and Administrative Expenses

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

General and administrative expenses were $5.6 million for the three months ended September 30, 2020, compared to $4.4 million for the three months ended September 30, 2019. General and administrative expenses were $15.6 million for the nine months ended September 30, 2020, compared to $13.7 million for the nine months ended September 30, 2019. The increase in general and administrative expenses in each period was primarily due to an increase in general and administrative personnel as well as increases in stock-based compensation. These increases were partially offset by a reduction in outside consulting expenses. We expect general and administrative expenses will grow during 2020 as we continue to build out the general and administrative functions.

Loss on Transfer of Nonfinancial Assets

During the three months ended September 30, 2019, we entered into contractual arrangements with two third parties that transferred the rights to develop and commercialize the programs underlying in-process research and development, or IPR&D, intangible assets acquired in our reverse merger with Arsanis, or the Merger (see Note 1 to the condensed consolidated financial statements). As a result of the transfer of control of the IPR&D projects to third parties, we recorded a charge of $4.0 million to “loss on transfer of non-financial assets" during the third quarter of 2019. The loss included the carrying value of the IPR&D intangible assets prior to the transfer of control, partially offset by nonrefundable, upfront fees received from the third parties.

In accordance with the contractual arrangements with these third parties, we are entitled to future fees that are contingent on the success of the third parties in advancing the applicable IPR&D projects toward commercialization. There was no such loss in the three or nine months ended September 30, 2020.

Other Expense, Net

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2020	2019	Change	2020	2019	Change
	(in thousands)			(in thousands)		
Interest income	$ —	$ 464	$ (464)	$ 272	$ 927	$ (655)
Interest expense	(697)	(688)	(9)	(1,968)	(1,599)	(369)
Change in fair value of preferred stock warrant and derivative liabilities	—	—	—	—	(105)	105
Loss on extinguishment of debt	—	(566)	566	(162)	(566)	404
Other income	228	52	176	494	201	293
Total other expense, net	$ (469)	$ (738)	$ 269	$ (1,364)	$ (1,142)	$ (222)

Other expenses, net, for the three months ended September 30, 2020 were lower as compared to the same period in the prior year due to losses on the extinguishment of debt in the prior period that did not recur in the current period, partially offset by lower interest income due to a general decline in interest rates on our cash equivalent investments. Other expenses, net, for the nine months ended September 30, 2020 were higher as compared to the same period in the prior year due to a reduction in interest income as a result of a general decline in interest rates on our cash equivalent investments and an increase in interest expense on our borrowings due to higher average outstanding borrowings on our Hercules facility.

Provision for Income Taxes

For the three months ended September 30, 2020, we did not record an income tax benefit for our losses as we have determined that a full valuation allowance is required on our net deferred tax assets in all jurisdictions. For the nine months ended September 30, 2020, we recorded a $0.1 million income tax provision related to local withholdings related to a milestone payment received from a customer in a foreign jurisdiction. No income tax benefit or expense was recorded in the prior year periods due to requirement for a full valuation allowance against our net deferred tax assets in all jurisdictions.

Liquidity and Capital Resources

Source of Liquidity

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. 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. In 2019, we completed a merger with Arsanis and acquired $26.4 million of its cash, cash equivalents and restricted cash. In addition, in 2019, we raised $139.4 million, net of offering costs, through public offerings of common stock, prefunded warrants to purchase shares of common stock, and accompanying warrants to purchase shares of common stock. In August 2020, we entered a Controlled Equity Offering^{SM} Sales Agreement with with B. Riley Securities, Inc., Cantor Fitzgerald & Co., and Stifel, Nicolaus & Company, Incorporated, or collectively, the Sales Agents, pursuant to which we may offer and sell, at our sole discretion through one or more of the Sales Agents, shares of our common stock having an aggregate offering price of up to $50.0 million. In October 2020, we entered into a common stock purchase agreement with Aspire Capital LLC, or Aspire Capital, pursuant to which Aspire Capital has committed to purchase, at our request from time to time over a 36-month period, shares of our common stock having an aggregate offering price of up to $50.0 million, subject to certain limitations.

As of September 30, 2020, our cash and cash equivalents were $88.8 million, and our restricted cash balance was $1.9 million. We believe that our current liquidity will be sufficient to meet our projected operating, investing and debt service requirements for at least the next twelve months from the issuance of these condensed consolidated financial statements. We expect that our research and development and general and administrative expenses will continue to increase and, as a result, we will need additional capital to fund our future operations, which we may raise through a combination of equity offerings, debt financings, other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements. The COVID-19 pandemic continues to rapidly evolve and has already resulted in a significant disruption of

global financial markets. If the disruption persists and deepens, we could experience an inability to access additional capital when and if needed. If we are unable to obtain funding, we could be forced to delay, reduce or eliminate some or all of our research and development programs, product portfolio expansion or commercialization efforts, which would adversely affect our business prospects, or we may be unable to continue operations.

Loan Facility with Hercules Capital, Inc.
In March 2020 and June 2019, we received $4.9 million and $9.8 million, respectively, in net proceeds as a result of refinancing our loan facility with Hercules Capital Inc., or Hercules. Under this facility, as amended most recently in March 2020, we have borrowed $25.0 million in term loans to date and may borrow an additional $15.0 million in term loans upon the achievement of certain operational milestones through June 2022 and an additional $10.0 million in term loans through December 2022, at the sole discretion of Hercules. Principal payments under the loan facility with Hercules commence in February 2022 and the agreement matures in July 2023.

Boston Lease
On November 11, 2019, we entered into a lease agreement for approximately 28,000 square feet of office space in a building located in Boston, Massachusetts. The office space has replaced our previous headquarters located in Cambridge, Massachusetts. We have begun to move into the premises under a phased approach compliant with COVID-19 guidance and protocols. Monthly rent payments under the lease commenced in May 2020, reflecting a 180-day rent-free period following the execution of the lease, and will be approximately $1.0 million annually, plus certain operating expenses, over the 7-year term of the lease. Substantially all constructions costs have been incurred as of September 30, 2020 and are reflected as a component of the Boston Lease right-of-use asset or building improvements on our condensed consolidated balance sheet. We expect to settle the remaining accrued expenses and accounts payable of $1.9 million related to the construction during the fourth quarter of 2020.

Cash Flows

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

	Nine Months Ended September 30,	
	2020	2019
	(in thousands)	
Net loss	$ (43,731)	$ (41,970)
Adjustments to reconcile net loss to net cash used in operating activities	5,198	7,132
Changes in operating assets and liabilities	(2,781)	(3,976)
Net cash used in operating activities	(41,314)	(38,814)
Net cash (used in) provided by investing activities	(1,060)	27,211
Net cash provided by financing activities	4,765	80,377
Effect of exchange rate changes on cash, cash equivalents and restricted cash	240	(266)
Net (decrease) increase in cash, cash equivalents and restricted cash	(37,369)	68,508
Cash, cash equivalents and restricted cash, beginning of period	$ 128,086	$ 8,498
Cash, cash equivalents and restricted cash, end of period	$ 90,717	$ 77,006

Operating Activities During the nine months ended September 30, 2020, net cash used in operating activities was $41.3 million, primarily resulting from our net loss of $43.7 million, adjusted for noncash expenses of $5.2 million and changes in our operating assets and liabilities of $2.8 million. Noncash expenses primarily includes stock-based compensation expense and non-cash interest expense. Net cash used in changes in our operating assets and liabilities primarily consisted of an increase in prepaid expenses due to the timing of payments related to our outsourced clinical trials and an increase in operating lease prepayments associated with our leased facility in Boston, Massachusetts.

Net cash used in operating activities for the nine months ended September 30, 2019 was $38.8 million, primarily resulting from our net losses of $42.0 million, adjusted for noncash expenses of $7.1 million and changes in our operating assets and liabilities of $4.0 million. Noncash expenses primarily include the derecognition of IPR&D intangible assets included within “loss on transfer of non-financial assets” and stock-based compensation expense. Net cash used in changes in our operating assets and liabilities primarily consisted of a decrease in accounts payable and accrued expenses as a result of the timing of cash settlement of these liabilities.

Investing Activities During the nine months ended September 30, 2020, cash used in investing activities of $1.1 million related primarily to furniture and laboratory equipment purchases related to our leased facility in Boston, Massachusetts, and our research and development center in Vienna, Austria. During the nine months ended September 30, 2019, net cash provided by investing activities consisted primarily of $26.4 million of cash and restricted cash acquired in connection with the Merger.

Financing Activities During the nine months ended September 30, 2020, net cash provided by financing activities was $4.8 million, consisting primarily of proceeds from the refinancing of our loan facility with Hercules in the first quarter of 2020. During the nine months ended September 30, 2019, net cash provided by financing activities was $80.4 million, consisting primarily of $79.3 million of net proceeds for the sale in April 2019 of our common stock, pre-funded warrants, and Class A warrants, and net proceeds of $9.8 million received in connection with the closing of our Amended and Restated Loan Agreement with Hercules in June 2019, partially offset by the settlement of loans acquired in the Merger.

Funding Requirements

We expect our expenses to increase in connection with our ongoing activities, particularly as we advance the clinical trials of our product candidates in development. In addition, we expect to continue to incur additional costs operating as a public company. 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 working capital requirements. Our future funding requirements will depend on and could increase significantly as a result of many factors, including:

- the scope, number, initiation, progress, timing, costs, design, duration, any potential delays, and results of clinical trials and nonclinical studies for our current or future product candidates, particularly our Phase 3 pivotal clinical trial of mavorixafor for the treatment of patients with WHIM syndrome, our Phase 1b clinical trial of mavorixafor in SCN and our Phase 1b clinical trial of mavorixafor in Waldenström's;
- diversion of healthcare resources away from the conduct of clinical trials as a result of the ongoing COVID-19 pandemic, including the diversion of hospitals serving as our clinical trial sites and hospital staff and independent physicians supporting the conduct of our clinical trials;
- the interruption of key clinical trial activities, such as clinical trial site monitoring, due to limitations on travel, quarantines or social distancing protocols imposed or recommended by federal or state governments, employers and others in connection with the ongoing COVID-19 pandemic;
- the clinical development plans 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;
- the cost of filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights covering our product candidates, including any such patent claims and intellectual property rights that we have licensed from Genzyme pursuant to the terms of our license agreement with Genzyme;
- our ability to maintain, expand and defend the scope of our intellectual property portfolio, including the cost of defending intellectual property disputes, including patent infringement actions brought by third parties against us or our product candidates;
- the cost and timing of completion of commercial-scale outsourced 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;
- the costs to continue to operate as a public company, including the need to implement additional financial and reporting systems and other internal systems and infrastructure for our business;
- market acceptance of our product candidates, to the extent any are approved for commercial sale; and
- the effect of competing technological and market developments.
- the ongoing impact of the COVID-19 pandemic on our operations.

We expect that our existing cash and cash equivalents will be sufficient to fund our operating expenses, capital expenditure requirements and debt service payments for at least the next twelve months. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our capital resources sooner than we expect.

Until such time, if ever, as we can generate substantial product revenue, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, and marketing, distribution or licensing arrangements with third parties. The COVID-19 pandemic continues to rapidly evolve and has already resulted in a significant disruption of global financial markets. If the disruption persists and deepens, we could experience an inability to access additional capital, which could in the future negatively affect our operations. To the extent that we raise additional capital through future equity offerings or debt financings, the ownership interest of our stockholders may be materially diluted, and the terms of such securities could include liquidation or other preferences that adversely affect the rights of our stockholders. Debt financing and preferred equity financing, if available, may involve agreements that include restrictive covenants that limit our ability to take specified actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings or other arrangements when needed, we may be required to delay, reduce or eliminate our product development efforts or future commercialization efforts, or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Loan and Security Agreements with Hercules Capital, Inc.

In October 2018, we entered into a Loan and Security Agreement, as amended, or the Hercules Loan Agreement, with Hercules, pursuant to which we borrowed $10.0 million under a term loan. In June 2019, we refinanced the Hercules Loan Agreement, as amended, and entered into an Amended and Restated Loan and Security Agreement, or the Amended and Restated Loan Agreement, with Hercules. The Amended and Restated Loan Agreement provided for aggregate maximum borrowings of $35.0 million, of which $10.0 million was previously outstanding and $10.0 million in new borrowings. On March 13, 2020, we entered into a First Amendment to the Amended and Restated Loan and Security Agreement, or the Amended Loan Agreement, with Hercules, which provides for aggregate maximum borrowings of up to $50.0 million. The Amended Loan Agreement provides for the following borrowing abilities:

(i) a term loan of $25.0 million (including the $20.0 million previously outstanding under the Amended and Restated Loan Agreement) and an additional $5.0 million drawn at the closing of the first amendment on March 13, 2020;

(ii) subject to the achievement of certain performance milestones and other conditions, we have the right to request that Hercules make additional term loan advances in an aggregate amount of up to $7.5 million through June 30, 2021;

(iii) subject to the achievement of certain performance milestones and conditions, we have the right to request that Hercules make additional term loan advances in an aggregate amount of up to $7.5 million through June 30, 2022; and

(iv) subject to Hercules investment committee's sole discretion, we have the right to request that Hercules make additional term loan advances in an aggregate amount of up to $10.0 million through December 31, 2022.

Borrowings under the Amended Loan Agreement bear interest at a variable rate equal to a per annum rate of interest equal to the greater of either (i) 3.75% plus the prime rate as reported in *The Wall Street Journal*, and (ii) 8.75%. In an event of default, as defined in the Amended Loan Agreement, and until such event is no longer continuing, the interest rate applicable to borrowings under the Amended Loan Agreement would be increased by 4.0%.

Borrowings under the Amended Loan Agreement are repayable in monthly interest-only payments through January 1, 2022, and in equal monthly payments of principal and accrued interest from February 1, 2022 until the maturity date of the loan, which is July 1, 2023. We may prepay all, but not less than all, of the outstanding borrowings, subject to a prepayment premium of up to 2.0%, 1.0% or 0.5% of the principal amount outstanding as of the date of repayment, in each case depending on when

such repayment is made. In addition, the Amended Loan Agreement provides for payments of (i) $0.8 million payable upon the earlier of November 1, 2021 or the repayment in full of all obligations under the Amended Loan Agreement, and (ii) 4.0% of the aggregate principal amount of all Term Loan Advances (as defined in the Amended Loan Agreement) drawn under the Amended Loan Agreement, payable upon the earlier of the maturity of the Amended Loan Agreement or the repayment in full of all obligations under the Amended Loan Agreement.

Borrowings under the Amended Loan Agreement are collateralized by substantially all of our personal property and other assets except for intellectual property (but including rights to payment and proceeds from the sale, licensing or disposition of the intellectual property). Under the Amended Loan Agreement, we have agreed to affirmative and negative covenants to which it will remain subject until maturity or repayment of the loan in full. The covenants include, without limitation:

(a) Effective immediately upon the date the outstanding principal amount of the advances under the Amended Loan Agreement exceeds $25.0 million, which has not yet occurred, we at all times thereafter shall maintain cash in an account or accounts in which Hercules has a first priority security interest, in an aggregate amount greater than or equal to the greater of (i) $30.0 million or (ii) 6 multiplied by a metric based on prior months' cash expenditures, or RML; provided, however, that from and after our achievement of certain performance milestones, the required level shall be reduced to the greater of (x) $20.0 million, or (y) 3 multiplied by the current RML; and provided further, that subject to the achievement of certain milestones, this covenant shall be extinguished.

(b) Restrictions on our 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.

Our obligations under the Amended Loan Agreement are subject to acceleration upon occurrence of specified events of default, including payment default, insolvency and a material adverse change in our business, operations or financial or other condition. In addition, under the Amended Loan Agreement, Hercules has the right to participate, subject to exceptions as provided in the Amended Loan Agreement, in a cumulative amount of up to $3.0 million in the aggregate, of which $1.0 million has already been exercised, in any future offering of our equity securities for cash on the same terms, conditions and pricing that is afforded to the other investors, where such future equity offering is solely for financing purposes and is broadly marketed to multiple investors.

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 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 nine months ended September 30, 2020, there were no material changes to our critical accounting policies as reported for the year ended December 31, 2019 as part of our Annual Report. In addition, see Note 2 of our consolidated financial statements under the heading "Recent Accounting Pronouncements" for new accounting pronouncements or changes to the accounting pronouncements during the nine months ended September 30, 2020.

Emerging Growth Company and Smaller Reporting Company Status

We are an "emerging growth company," or EGC, as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. The JOBS Act permits an EGC to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have irrevocably elected to "opt out" of this provision and, as a result, we will comply with new or revised accounting standards when they are required to be adopted by public companies that are not EGCs.

In addition, we are also a smaller reporting company as defined in the Exchange Act. We may continue to be a smaller reporting company even after we are no longer an emerging growth company. We may take advantage of certain of the scaled

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

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

Item 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

As a smaller reporting company, as defined by Rule 12b-2 of the Exchange Act and in Item 10(f)(1) of Regulation S-K, we are electing scaled disclosure reporting obligations and therefore 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 Securities Exchange Act of 1934, as amended, or 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, 2020, and have concluded that, based on such evaluation, our disclosure controls and procedures were effective as of September 30, 2020 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, 2020 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.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred significant losses 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-stage clinical biopharmaceutical company with a limited operating history. Since inception, we have incurred significant operating losses. Without regard to the historical operating results of our predecessor, Arsanis, our net losses were $52.8 million, $33.3 million and $22.0 million for the years ended December 31, 2019, 2018 and 2017, respectively, our net losses were $43.7 million for the nine months ended September 30, 2020, and we had an accumulated deficit of $175.8 million as of September 30, 2020. To date, we have financed our operations primarily through issuances of shares 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. In 2019, we completed a merger with Arsanis and acquired its $26.4 million of cash, cash equivalents and restricted cash. Since inception, we have devoted substantially all of our efforts to research and preclinical and clinical development of our product candidates, as well as to expanding our infrastructure. We have not generated any revenue from product sales to date. It could be several years, if ever, before we have a commercialized drug. The net losses we incur may fluctuate significantly from quarter to quarter and year to year. We expect to continue to incur significant expenses and increasing operating losses for at least the next few years as we conduct additional clinical trials for our product candidates; continue to discover and develop additional product candidates; acquire or in-license other product candidates and technologies; maintain, expand and protect our intellectual property portfolio; hire additional clinical, scientific and commercial personnel; establish a commercial manufacturing source and secure supply chain capacity sufficient to provide commercial quantities of any product candidates for which we may obtain regulatory approval; seek regulatory approvals for any product candidates that successfully complete clinical trials; establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain regulatory approval; and add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. For example, we have already experienced delays in clinical trial site activation and slower patient enrollment in some of our clinical trials as a result of the pandemic, which we expect will delay our ability to report data from those trials, and we may encounter additional delays, disruptions and other direct and indirect negative effects of the COVID-19 pandemic on our clinical trials. 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. Even if we achieve profitability in the future, we may not be able to sustain profitability in subsequent periods. The net losses we incur may fluctuate significantly from quarter to quarter and year to year.

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

- 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 3 trial of mavorixafor for the treatment of WHIM syndrome, our Phase 1b clinical trial of mavorixafor for the treatment of severe congenital neutropenia, or SCN, and our Phase 1b clinical trial of mavorixafor for the treatment of Waldenström’s macroglobulinemia.

- 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 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; and
- the number and characteristics of product candidates and programs that we pursue.

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

We may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors that may alter or delay our plans. Assuming that we complete the development of and obtain marketing approval for any of our product candidates, 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 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.

Our operations have consumed a large amount of cash since inception. 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 United States 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, 2020, our cash and cash equivalents was $88.8 million, which we believe will fund our current operating plans through at least the next 12 months from the date the condensed consolidated financial statements were issued. However, our operating plan may change as a result of many factors currently unknown to us, and we may need to seek additional funds sooner than planned, through public or private equity or debt financings, third-party funding, marketing and distribution arrangements, as well as other collaborations, strategic alliances and licensing arrangements, or any combination of these approaches.

We will require substantial additional funding to carry out our business plans, including the clinical development of mavorixafor. Further, even if we believe we have sufficient capital for our current or future operating plans, we may seek additional capital if market conditions are favorable or if we have specific strategic considerations. We cannot be certain that additional funding will be available on acceptable terms, or at all. The ongoing COVID-19 pandemic has already resulted in a significant disruption of global financial markets. If the disruption persists and deepens, we could experience an inability to access additional capital when and if needed. 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.

We also could be required to:

- seek 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, particularly our Phase 3 trial of mavorixafor for the treatment of WHIM syndrome, our Phase 1b clinical trial of mavorixafor for the treatment of SCN, and our Phase 1b clinical trial of mavorixafor for the treatment of Waldenström's;
- 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 U.S. Food and Drug Administration, or FDA, and comparable foreign regulatory authorities, including the potential for the FDA or comparable foreign regulatory authorities to require that we perform more studies for our product candidates than those that we currently expect;
- our ability to obtain marketing approval for our product candidates;
- the cost of filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights covering our product candidates, including any such patent claims and intellectual property rights that we have licensed from Genzyme pursuant to the terms of our license agreement with Genzyme 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 outsourced 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;
- the costs to operate as a public company, including the need to implement additional financial and reporting systems and other internal systems and infrastructure for our business;
- market acceptance of our product candidates, to the extent any are approved for commercial sale;
- the effect of competing technological and market developments; and
- business interruptions resulting from pandemics and public health emergencies, including those related to the ongoing COVID-19 pandemic, geopolitical actions, including war and terrorism or natural disasters including earthquakes, typhoons, floods and fires.

If we do not raise additional capital in sufficient amounts, or on terms acceptable to us, we may be prevented from pursuing discovery, development and commercialization efforts, which will harm our business, operating results and prospects.

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. The COVID-19 pandemic has already resulted in a significant disruption of global financial markets. If the disruption persists and deepens, we could experience an inability to access additional capital. Other than our common stock purchase agreement with Aspire Capital, pursuant to which Aspire Capital is obligated, subject to certain limitations, to purchase up to $50.0 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 our assets and intellectual property. If we default on such indebtedness, we could lose such assets and intellectual property.

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 any revenues from product sales since inception and may never become profitable.

To date, we have not generated any revenues from any product sales. Our ability to generate revenue and become profitable depends upon our ability to successfully obtain marketing approval and commercialize our product candidates, including mavorixafor, X4P-002, X4P-003 or other product candidates that we may develop, in-license or acquire in the future. Even if we are able to successfully achieve regulatory approval for these product candidates, we are unable to predict the extent of any future losses and do not know when any of these product candidates will generate revenue for us, if at all. Our ability to generate revenue from mavorixafor or any of our current or future product candidates also depends on a number of additional factors, including our ability to:

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

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

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

Risks Related to Development of Our Product Candidates

We depend almost entirely on the success of our lead product candidate, mavorixafor, which we are developing initially for the treatment of WHIM syndrome, for the treatment of SCN, for the treatment of Waldenström's and, with a potential strategic partner, for the treatment of ccRCC. We cannot be certain that we will be able to obtain regulatory approval for, or successfully commercialize, mavorixafor or any other product candidate.

Our business depends almost entirely on the successful clinical development, regulatory approval and commercialization of mavorixafor. We currently have no products for sale and may never be able to develop marketable drug products. We initiated a

global Phase 3 pivotal clinical trial of our lead product candidate, mavorixafor, in WHIM patients in the second quarter of 2019, and may be required to complete additional nonclinical studies and clinical trials before we can seek regulatory approval. In the fourth quarter of 2019, we also initiated Phase 1b clinical trials of mavorixafor for the treatment of SCN and Waldenström's. While we completed a Phase 2a clinical trial of mavorixafor for the treatment of ccRCC, we do not plan to develop mavorixafor for this indication on our own. Our other programs, including X4P-002 and X4P-003, are still in the preclinical development stage. The clinical trials of our product candidates are, and the manufacturing and marketing of our product candidates will be, subject to extensive and rigorous review and regulation by government authorities in the United States and in other countries where we intend to test and, if approved, market any product candidate. Before obtaining regulatory approvals for the commercial sale of any product candidate, we must successfully meet a number of critical developmental milestones, including:

- developing dosages that will be well-tolerated, safe and effective;
- completing the development and scale-up to permit manufacture of our product candidates in commercial quantities and at acceptable costs;
- demonstrating through pivotal clinical trials that each product candidate is safe and effective in patients for the intended indication;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers; and
- obtaining and maintaining patent and trade secret protection and non-patent exclusivity for our product candidates.

The time necessary to achieve these developmental milestones for any individual product candidate is long and uncertain, and we may not successfully complete these milestones for mavorixafor or any other product candidates that we may develop. We 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, X4P-002 for the treatment of glioblastoma multiforme, or GBM, and X4P-003, a next generation molecule for the treatment of rare diseases linked to defects in CXCR4 trafficking.

We are continuing to test and develop our product candidates and may explore possible design or formulation changes to address safety, efficacy, manufacturing efficiency and performance issues to the extent any arise. We may not be able to complete development of any product candidates that demonstrate safety and efficacy and that will have a commercially reasonable treatment and storage period. If we are unable to complete development of mavorixafor or any other product candidates that we may develop, we will not be able to commercialize and earn revenue from them.

We expect to develop mavorixafor, and potentially future product candidates, in combination with other therapies, which exposes us to additional risks.

We intend to develop mavorixafor, and may develop future product candidates, in combination with one or more currently approved cancer therapies. Even if any product candidate we develop were to receive marketing approval or be commercialized for use in combination with other existing therapies, we would continue to be subject to the risks that the FDA or similar regulatory authorities outside of the United States could revoke approval of the therapy used in combination with our product candidate or that safety, efficacy, manufacturing or supply issues could arise with these existing therapies. Combination therapies are commonly used for the treatment of cancer, and we would be subject to similar risks if we develop any of our product candidates for use in combination with other drugs or for indications other than cancer. 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 cancer 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 mavorixafor, our business will be substantially harmed.

Of the large number of drugs in development in the United States, only a small percentage receive FDA regulatory approval and are commercialized in the United States. 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 the marketing authorization application, or MAA, in the European Union from the European Medicines Agency, or EMA. Prior to submitting an NDA to the FDA for approval of mavorixafor for the treatment of WHIM syndrome, we will need to successfully complete our current Phase 3 pivotal clinical trial of mavorixafor in patients with WHIM syndrome and potentially, we will 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 changes in the approval requirements that render its nonclinical and clinical data insufficient to support the filing of an NDA or to obtain regulatory approval; or
- changes in clinical practice in or approved products available for the treatment of the target patient population that could have an impact on the indications that we are pursuing for mavorixafor or our other product candidates.

The FDA or a comparable foreign regulatory authority may also require more information, including additional nonclinical or clinical data to support approval, which may delay or prevent approval of our commercialization plans, or cause us to abandon the development program. Even if we obtain regulatory approval, our product candidates may be approved for fewer or more limited indications than we request, such approval may be contingent on the performance of costly post-marketing clinical trials, or we may not be allowed to include the labeling claims necessary or desirable for the successful commercialization of such product candidate. 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 and Georgetown University 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 and Georgetown University under which we were granted rights to patents and patent applications that are important to our business. We rely on these license agreements in order to be able to use various proprietary technologies that are material to our business, including certain patents and patent applications that cover our product candidates, including mavorixafor. Our rights to use these patents and patent applications and employ the inventions claimed in these licensed patents are subject to the continuation of and our compliance with the terms of our license agreements.

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

- our obligation to pay Genzyme milestone payments in the aggregate amount of up to $25.0 million, contingent upon our achievement of certain late-stage regulatory and sales milestones with respect to licensed products.
- our obligation to pay Genzyme tiered royalties based on net sales of licensed products that we commercialize under the agreement.
- our obligation to pay Genzyme a certain percentage of cash payments received by us or our affiliates in consideration for the grant of a sublicense under the license granted to us by Genzyme.

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

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

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

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

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

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

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

Furthermore, certain of the above risks and uncertainties may be amplified as a result of the impact of COVID-19. The extent to which COVID-19 may impact our license agreements with Genzyme, Beth Israel Deaconess Medical Center and/or Georgetown University, or any other third-party partner, will depend on future developments, which are highly uncertain and cannot be predicted, including new information which may emerge concerning the severity of COVID-19 and the actions to contain COVID-19 or treat its impact, among others.

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

Delays in 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.

We may experience delays in our current or future clinical trials, including our Phase 3 trial of mavorixafor for the treatment of WHIM syndrome, our Phase 1b clinical trial of mavorixafor for the treatment of SCN, and our Phase 1b clinical trial of mavorixafor for the treatment of Waldenström's. As a result of the ongoing COVID-19 pandemic, we have already experienced delays in clinical trial site activation and slower patient enrollment in our clinical trials of mavorixafor for the treatment of WHIM syndrome and SCN. We do not know whether planned clinical trials will begin or enroll subjects on time, need to be redesigned or be completed on schedule, if at all. Clinical trials may be delayed, suspended or terminated for a variety of reasons, including the following:

- direct and indirect effects of the ongoing COVID-19 pandemic on various aspects and stages of the clinical development process, including the potential impact to expected site activation, enrollment and participation in our clinical trials;
- the diversion of healthcare resources away from the conduct of clinical trials as a result of the ongoing COVID-19 pandemic, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials;
- the interruption of key clinical trial activities, such as clinical trial site monitoring, due to limitations on travel, quarantines or social distancing protocols imposed or recommended by federal or state governments, employers and others in connection with the ongoing COVID-19 pandemic;
- delay or failure in reaching agreement with the FDA or a comparable foreign regulatory authority on a trial design that we are able to execute;
- delay or failure in obtaining authorization to commence a trial or inability to comply with conditions imposed by a regulatory authority regarding the scope or design of a clinical trial;
- inability, delay or failure in identifying and maintaining a sufficient number of trial sites, many of which may already be engaged in competing clinical trial programs;
- delay or failure in recruiting and enrolling suitable subjects to participate in a trial;
- delay or failure in having subjects complete a trial or return for post-treatment follow-up;
- clinical sites and investigators deviating from trial protocol, failing to conduct the trial in accordance with regulatory requirements, or dropping out of a trial;
- delay or failure in reaching agreement on acceptable terms with prospective clinical research organizations, or 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, or IRB, approval to conduct a clinical trial at each site;
- delays resulting from negative or equivocal findings of the Data Safety Monitoring Board, or 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 drug product for use in nonclinical studies or clinical trials;
- lack of adequate funding to continue the product development program; or
- changes in governmental regulations or requirements.

Any delays in completing 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.

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 and enroll 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 ongoing COVID-19 pandemic, we have experienced, and expect to continue to experience, enrollment at a slower pace at certain of our clinical trial sites than initially expected. In addition, certain of our clinical trial sites have suspended enrollment due to facility closures, quarantine, travel restrictions and other governmental restrictions. As a result, we expect the results from our clinical trials of mavorixafor for the treatment of WHIM syndrome, SCN and Waldenström's to be delayed, which we expect will have a material adverse impact on our clinical trial plans and timelines.

If we cannot identify patients to participate in our clinical trials, whether due to COVID-19 or otherwise, 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, including mavorixafor, X4P-002 or X4P-003, 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 clinical trials in a timely manner. In particular, we are currently evaluating mavorixafor for the treatment of WHIM syndrome, SCN and Waldenström's, 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.

Patient enrollment, a significant factor in the duration of clinical trials, is also affected by many factors, including:

- delays or difficulties in clinical site activation, including difficulties in training clinical site investigators and clinical site staff;
- diversion or prioritization of healthcare resources away from the conduct of our clinical trials as a result of the ongoing COVID-19 pandemic, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials, particularly for clinical trials that require in-patient monitoring following administration of the product candidate;
- increased rates of patients withdrawing from our clinical trials following enrollment as a result of contracting COVID-19, being forced to quarantine, or being unable to visit clinical trial locations;
- the severity of the disease under investigation;
- the size and nature of the patient population (particularly with respect to orphan drugs which, by definition, are intended for a relatively small patient population);
- the eligibility criteria for the clinical trial in question;
- the design of the clinical trial;
- the inability to obtain and maintain patient consents;
- the risk that enrolled subjects will drop out before completion;
- clinicians' and patients' perceptions as to the potential advantages of the drug being studied in relation to other available therapies, including any new drug that may be approved or for which clinical trials are initiated for the indications that we are investigating;
- our CROs and our trial sites' efforts to facilitate timely screening and enrollment in clinical trials and while we will have agreements governing their activities, we have limited control over their actual performance;
- patient referral practices of physicians; and
- our ability to monitor patients adequately during and after treatment, which may be affected by COVID-19.

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 in WHIM syndrome, SCN or Waldenström's 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.

If the size of the commercial opportunities in any of our target indications is smaller than we anticipate, we may not be able to achieve profitability and growth. We are developing mavorixafor initially as a treatment for patients with WHIM syndrome and also as a treatment for other rare diseases, including primary immunodeficiencies such as SCN and cancer such as Waldenström's. WHIM syndrome, SCN and Waldenström's each have a limited patient population.

For example, we are 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 have updated our estimates and we now estimate there are between 1,800 and 3,700 WHIM patients in the United States, many of which were previously undiagnosed. If the commercial opportunity in 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, SCN and Waldenström's. Our projections of the number of people who have WHIM syndrome (or its other potential primary immunodeficiencies), SCN or Waldenström's 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.

If we experience any of a number of possible unforeseen events in connection with our clinical trials, potential marketing approval or commercialization of our product candidates, or our entry into licensing, collaboration or similar arrangements, could be delayed or prevented.

We may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize our product candidates, including:

- diversion or prioritization of healthcare resources away from the conduct of clinical trials as a result of the ongoing COVID-19 pandemic, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials;
- the interruption of key clinical trial activities, such as clinical trial site monitoring, due to limitations on travel, quarantines or social distancing protocols imposed or recommended by federal or state governments, employers and others in connection with the ongoing COVID-19 pandemic;
- clinical trials of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon product development programs;
- the number of patients required for clinical trials of our product candidates may be larger than we anticipate, enrollment in these clinical trials may be slower than we anticipate or participants may drop out of these clinical trials at a higher rate than we anticipate;
- we may be unable to recruit and enroll a sufficient number of patients in our clinical trials to ensure adequate statistical power to detect any statistically significant treatment effects;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- regulators, institutional review boards or independent ethics committees may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we may experience delays in reaching, or we may fail to reach, agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;
- we may have to suspend or terminate clinical trials of our product candidates for various reasons, including a finding that the participants are being exposed to unacceptable health risks or undesirable side effects;
- regulators, institutional review boards or independent ethics committees may require that we or our investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- the cost of clinical trials of our product candidates may be greater than we anticipate;

- the supply or quality of our product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient or inadequate; and
- our product candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators, institutional review boards or independent ethics committees to suspend or terminate the clinical trials.

Our product development costs will increase if we experience delays in testing or marketing approvals. We do not know whether any preclinical tests or clinical trials will begin as planned, will need to be redesigned or will be completed on schedule, or at all. We have already experienced delays in clinical trial site activation and slower patient enrollment in our Phase 3 clinical trial of mavorixafor in patients with WHIM syndrome and our Phase 1b clinical trial of mavorixafor in patients with SCN as a result of the ongoing COVID-19 pandemic. Significant preclinical study or clinical trial delays, including as a result of COVID-19, also could shorten any periods during which we may have the exclusive right to commercialize our product candidates, if they are approved, or allow our competitors to bring products to market before we do and impair our ability to successfully commercialize our product candidates, which may harm our business and results of operations.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus on specific product candidates. Currently, we are focusing our resources predominantly on the development mavorixafor for the treatment of WHIM syndrome, for the treatment of SCN and for the treatment of Waldenström's. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that have or that could later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or alternate and/or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

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

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

We have obtained both breakthrough therapy and Fast Track designations for mavorixafor for the treatment of adult patients with WHIM and we may pursue those designations for other product candidates as well. A breakthrough therapy is defined as a product that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening condition, and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. For product candidates that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Such designation also offers an intensive and efficient review involving FDA senior managers and experienced review and regulatory health project management staff across disciplines. 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. Even if we do apply for and receive Fast Track designation, we may not experience a faster development, review or approval process compared to conventional FDA procedures. The FDA may rescind Fast Track designation if it believes that the designation is no longer supported by data from our clinical development program.

Designation as a breakthrough therapy and Fast Track designation 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 receipt of either or both of a 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 or for Fast Track designation, the FDA may later decide that the products no longer meet the conditions for qualification.

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

Regulatory authorities in some jurisdictions, including the United States and Europe, 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, or 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. 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 the European Union allows 10 years of exclusivity in Europe. The European exclusivity period can be reduced to six years if a drug no longer meets the criteria for orphan drug designation or 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 under certain 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.

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 product candidates, if approved, among hospitals, physicians, patients and healthcare payors.

Even if we obtain regulatory approval for any of our product candidates that we may develop or acquire in the future, the product may not gain market acceptance among hospitals, physicians, health care payors, patients and the medical community. Market acceptance of any of our product candidates for which we receive approval depends on a number of factors, including:

- the efficacy and safety of such product candidates as demonstrated in clinical trials;
- the clinical indications for which the product candidate is approved;
- acceptance by 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 product candidates over alternative treatments as compared to the relative costs of the product candidates and alternative treatments;
- the prevalence and severity of any side effects with respect to our product candidates, including mavorixafor;
- our ability to offer any approved products for sale at competitive prices;
- the timing of market introduction of our products as well as competitive products;
- our pricing, and the availability of coverage and adequate reimbursement by third party payors and government authorities;
- relative convenience and ease of administration; and
- the effectiveness of our sales and marketing efforts and those of our potential future collaborators.

There may be delays in getting our product candidates, if approved, on hospital or insurance formularies or limitations on coverages that may be available in the early stages of commercialization for newly approved drugs. If any of our product candidates are approved but fail to achieve market acceptance among hospitals, physicians, patients or health care payors, we will not be able to generate significant revenues, which would have a material adverse effect on our business, prospects, financial condition and results of operations.

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.

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

If we obtain regulatory approval for a product candidate, it would be subject to extensive ongoing requirements by the FDA and comparable foreign regulatory authorities governing the manufacture, quality control, further development, labeling, packaging, storage, distribution, safety surveillance, import, export, advertising, promotion, recordkeeping and reporting of safety and other post-market information. The safety profile and efficacy of any product will continue to be closely monitored by the FDA and comparable foreign regulatory authorities after approval. If the FDA or comparable foreign regulatory authorities become

aware of new safety information after approval of any of our product candidates, these regulatory authorities may require labeling changes or the FDA may require establishment of a Risk Evaluation Mitigation Strategy, or REMS, or similar strategy, impose significant restrictions on a product's indicated uses or marketing, impose ongoing requirements for potentially costly post-approval studies or post-market surveillance. Progress reports are required at quarterly intervals, every six months and at annual intervals depending upon the country, and more frequently if serious adverse events occur.

In addition, manufacturers of drugs and their facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP regulations. If a regulatory agency discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory agency may impose restrictions on that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing. If we, our product candidates or the manufacturing facilities for our product candidates fail to comply with cGMPs and other applicable regulatory requirements, the FDA may, among other things:

- issue warning letters;
- request modifications to promotional materials or require us to provide corrective information to healthcare practitioners;
- require us to enter into a consent decree, which can include imposition of various fines, reimbursements for inspection costs, required due dates for specific actions and penalties for noncompliance;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve pending applications or supplements to applications filed by us;
- suspend or impose restrictions on operations, including costly new manufacturing requirements; or
- seize or detain products, refuse to permit the import or export of products, or require us to initiate a product recall.

The occurrence of any event or penalty described above may inhibit our ability to commercialize our products and generate revenue.

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. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we do not market our products for their approved indications, we may be subject to enforcement action for off-label marketing and/or promotion.

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 sales and marketing capabilities or to selectively enter into agreements with third parties to sell and market our product candidates, we may not be successful in commercializing our product candidates if and when they are approved.

We do not have a sales or marketing infrastructure and have no experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for any approved product for which we retain sales and marketing responsibilities, we must either develop a sales and marketing organization or outsource these functions to other third parties. In the future, we may choose to build a focused sales and marketing infrastructure to sell some of our product candidates if and when they are approved.

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

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

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

If we enter into arrangements with third parties to perform sales, marketing and distribution services, our product revenue or the profitability of these product revenue to us may be lower than if we were to market and sell any products that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell and market our product candidates or may be unable to do so on terms that are favorable to us. We may have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates.

We face substantial competition, which 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. There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of cancer, such as ccRCC. 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.

Our lead product candidate, mavorixafor, is in clinical development for the treatment of WHIM syndrome, SCN and Waldenström's, respectively. We are aware of other companies that are developing CXCR4 inhibitors that are in a similar stage of development as mavorixafor, including Eli Lilly, Pfizer, Bristol-Myers Squibb, or BMS, 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 SCN. With respect to Waldenström's, the Dana Farber Cancer Institute has completed enrollment for a trial to study the BMS CXCR4 antibody (IV infusion) in the treatment of Waldenström's patients with CXCR4 mutations. In Waldenström's, there are several treatment approaches currently being developed, including targeted therapies and immunotherapies (as monotherapies and combination therapies), chemotherapy, stem cell transplantation, and cancer vaccines. With the exception of the BMS monoclonal antibody, none to our knowledge have a mechanism of action that interacts with the CXCR4 receptor.

There are a variety of available drug therapies marketed for cancer. In many cases, 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 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.

We will be required to obtain international regulatory approval to market and sell our product candidates outside of the United States.

We will be required to obtain international regulatory approval to market and sell our product candidates outside of the United States. In order to market and sell our products in the European Union and many other jurisdictions, we must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The regulatory review and approval process outside the United States generally includes all of the risks associated with obtaining FDA approval, but can involve additional testing and clinical trial requirements and in-country regulatory and/or legal representation. We may need to partner with third parties in order to obtain approvals outside the United States. In addition, in many countries worldwide, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any market. If we are unable to obtain approval of mavorixafor or any other product candidate by regulatory authorities in the European Union or other countries, the commercial potential of those product candidates may be significantly diminished and our business prospects could decline.

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 foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. If 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. 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, 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, such as the ongoing COVID-19 pandemic.

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

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

The laws and regulations that govern marketing approvals, pricing, coverage and reimbursement for new drug products vary widely from country to country. Current and future legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted and, in some markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval.

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

Government authorities and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that coverage and reimbursement will be available for mavorixafor or any other product that we commercialize and, if coverage and reimbursement is available, the level of reimbursement. Reimbursement may impact the demand for, or the price of, any

product candidate for which we obtain marketing approval. Obtaining and maintaining adequate reimbursement for mavorixafor may be particularly difficult because of the higher prices typically associated with drugs directed at smaller populations of patients. In addition, third-party payors are likely to impose strict requirements for reimbursement of a higher priced drug, and any launch of a competitive product is likely to create downward pressure on the price initially charged. If reimbursement is not available or is available only to a limited degree, we may not be able to successfully commercialize any product candidate for which we obtain marketing approval.

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

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

Governments outside the United States tend to impose strict price controls, which may adversely affect our revenues, if any.

In some countries, particularly the countries of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be harmed, possibly materially.

Product liability lawsuits against us could cause us to incur substantial liabilities and could limit the commercialization of any product candidates we may develop.

We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical trials and will face an even greater risk with respect to commercial sales of any products that we may develop. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- reduced resources of our management to pursue our business strategy;
- decreased demand for any products that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend any related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue;
- increased insurance costs; and
- the inability to commercialize any products that we may develop.

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

Risks Related to Government Regulation

Our relationships with customers and third-party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

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

- the federal Anti-Kickback Statute prohibits, among other things, persons from knowingly 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 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, or HIPAA, imposes criminal and civil liability for 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, or collectively the ACA, 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, or CMS information related to payments and other transfers of value to physicians, as defined by such law, and teaching hospitals and the ownership and investment interests of physicians and their immediate family members in such manufacturers, which will be expanded in 2022, to require applicable manufacturers to report such information regarding its relationships with physicians assistants, nurse practitioners, clinical nurse specialists, certified registered nurse anesthetists and certified nurse midwives during the previous year;
- 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 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 laws also govern the privacy 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.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to it, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment,

exclusion of products from government funded healthcare programs, such as Medicare and Medicaid, additional reporting requirements and/or oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws and the curtailment or restructuring of our operations. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to significant criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

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

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay marketing approval of our product candidates, restrict post-approval activities and affect our ability to sell profitably any product candidates for which we obtain marketing approval.

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 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 now 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 remain challenges to certain aspects of the ACA, and we expect there will be additional challenges and amendments to the ACA in the future. Since January 2017, President Trump has signed Executive Orders and other directives designed to eliminate the implementation of certain provisions of the ACA or otherwise circumvent some of the requirements for health insurance mandated by the ACA. Concurrently, Congress has considered legislation that would repeal or repeal and replace all or part of the ACA. While Congress has not passed comprehensive repeal legislation, several bills affecting the implementation of certain taxes under the ACA have been signed into law. The Tax Cuts and Jobs Act, or the Tax Act, was enacted, which, among other things, included a provision which repealed, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate". In addition, the 2020 federal spending package permanently eliminated, effective January 1, 2020, the ACA-mandated "Cadillac" tax on certain high-cost employer-sponsored health insurance plans and the medical device excise tax on non-exempt medical devices and, effective January 1, 2021, also eliminates the health insurer tax. On December 14, 2018, a Texas U.S. District Court Judge ruled that the ACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress as part of the Tax Act. Additionally, on December 18, 2019, the U.S. Court of Appeals for the 5th Circuit upheld the District Court ruling that that the individual

mandate was unconstitutional and remanded the case back to the District Court to determine whether the remaining provisions of the ACA are invalid as well. On March 2, 2020, the United States Supreme Court granted the petitions for writs of certiorari to review this case, which are expected to occur in the fall. It is unclear how this litigation and other efforts to repeal and replace the ACA will impact the ACA.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted. These new laws may result in additional reductions in Medicare and other healthcare funding.

There has also been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several Congressional inquiries and proposed 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, the Trump administration's budget proposal for fiscal year 2021 includes a $135 billion allowance to support legislative proposals seeking to reduce drug prices, increase competition, lower out-of-pocket drug costs for patients, and increase patient access to lower-cost generic and biosimilar drugs. On March 10, 2020, the Trump administration sent "principles" for drug pricing to Congress, calling for legislation that would, among other things, cap Medicare Part D beneficiary out-of-pocket pharmacy expenses, provide an option to cap Medicare Part D beneficiary monthly out-of-pocket expenses, and place limits on pharmaceutical price increases. Further, the Trump administration previously released a "Blueprint" to lower drug prices and reduce out of pocket costs of drugs that contained additional proposals to increase drug manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products, and reduce the out of pocket costs of drug products paid by consumers. The Department of Health and Human Services, or HHS, has solicited feedback on some of these measures and has implemented others under its existing authority. On July 24, 2020, President Trump announced four executive orders related to prescription drug pricing that attempt to implement several of the administration's proposals, including a policy that would tie certain Medicare Part B drug prices to international drug prices, the details of which were released on September 13, 2020 and also expanded the policy to cover certain Part D drugs; one that directs HHS to finalize the Canadian drug importation proposed rule previously issued by HHS and makes other changes allowing for personal importation of drugs from Canada; one that directs HHS to finalize the rule-making process on modifying the anti-kickback law safe harbors for plans, pharmacies, and pharmaceutical benefit managers; and one that reduces costs of insulin and epipens to patients of federally qualified health centers. The FDA also recently released a final rule, effective November 30, 2020, implementing a portion of the importation executive order providing guidance for states to build and submit importation plans for drugs from Canada. While some of these and other measures may require additional authorization to become effective. Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. Individual states in the United States have also become increasingly active in passing legislation and implementing regulations designed to control pharmaceutical 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 expect that the ACA, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we will receive for any approved product. It is possible that additional governmental action may be taken to address the COVID-19 pandemic. For example, the Coronavirus Aid, Relief and Economic Security Act, or CARES Act, which was signed into law in March 2020 and is designed to provide financial support and resources to individuals and businesses affected by the COVID-19 pandemic, suspended the 2% Medicare rate reduction sequester from May 1, 2020 through December 31, 2020, and extended the sequester by one year, through 2030. Further, on April 18, 2020, CMS announced that qualified health plan issuers under the ACA may suspend activities related to the collection and reporting of quality data that would have otherwise been reported between May and June 2020 given the challenges healthcare providers are facing responding to the COVID-19 virus. Further, on August 6, 2020, President Trump issued an executive order that instructs the federal government to develop a list of "essential" medicines and then buy them and other medical supplies from U.S. manufacturers instead of from companies around the world, including China. The order is meant to reduce regulatory barriers to domestic pharmaceutical manufacturing and catalyze manufacturing technologies needed to keep drug prices low and the production of drug products in the United States. Any reduction in payments from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our products.

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

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, or FCPA, and other anti-corruption laws that apply in countries where we do business and may do business in the future. The FCPA and these other laws generally prohibit us, our officers and employees and intermediaries from bribing, being bribed or making other prohibited payments to government officials or other persons to obtain or retain business or gain some other business advantage. We may in the future operate in jurisdictions that pose a high risk of potential FCPA violations, and may participate in collaborations and relationships with third parties whose actions could potentially subject us to liability under the FCPA or local anti-corruption laws. In addition, we cannot predict the nature, scope or effect of future regulatory requirements to which its international operations might be subject or the manner in which existing laws might be administered or interpreted.
We are also subject to other laws and regulations governing our international operations, including regulations administered by the U.S. government and authorities in the European Union or the United Kingdom, including applicable export control regulations, economic sanctions on countries and persons, customs requirements and currency exchange regulations, which we collectively refer to as Trade Control Laws.

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

Risks Related to Our Dependence on Third Parties

We have minimal 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, or 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 do not own or operate facilities for the manufacture of mavorixafor or any other product candidate. We currently have no plans to build our own clinical or commercial scale manufacturing capabilities. We currently work exclusively with one manufacturer for the production of mavorixafor, the active pharmaceutical ingredient, or API, and a single manufacturer of mavorixafor finished drug product capsules. We cannot guarantee that these third parties will continue to perform their contractual duties in a timely and satisfactory manner as a result of the COVID-19 pandemic, which could negatively impact our supply chain activities and our clinical supply.

To meet our projected needs for clinical supplies to support our activities through regulatory approval and commercial manufacturing, the manufacturer with whom we currently work will need to increase its frequency and/or scale of production or we will need to find additional or alternative manufacturers. We have not yet secured alternate suppliers in the event the current manufacturer we utilize is unable to meet demand, or if otherwise we experience any problems with them. If such problems arise and we are unable to arrange for alternative third-party manufacturing sources, we are unable to find an alternative third party capable of reproducing the existing manufacturing method or we are unable to do so on commercially reasonable terms or in a timely manner, we may not be able to complete development of our product candidates, or market or distribute them.
Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured our product candidates ourselves, including reliance on the third party for regulatory compliance and quality assurance, the possibility of breach of the manufacturing agreement by the third party because of factors beyond our control (including a failure to synthesize and manufacture our product candidates or any products that we may eventually commercialize in accordance with our specifications), and the possibility of termination or nonrenewal of the agreement by the third party, based on its own business priorities, at a time that is costly or damaging to us. In addition, the FDA and other regulatory authorities require that our product candidates and any products that we may eventually commercialize be manufactured according to cGMP and similar foreign standards. Drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and some state agencies, and are subject to periodic unannounced inspections for compliance with cGMP requirements. Changes to the manufacturing process are strictly regulated and often require prior FDA or other regulatory authority approval before being implemented. FDA requirements also require

investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon us and any third-party manufacturers that we may decide to use. Accordingly, the manufacturers must continue to expend time, money and effort in the areas of production and quality control to maintain cGMP compliance. Any failure by our third-party manufacturers to comply with cGMP or failure to scale up manufacturing processes, including any failure to deliver sufficient quantities of product candidates or products if they are approved in a timely manner, could lead to a delay in, or failure to obtain, regulatory approval of any of our product candidates. In addition, such failure could be the basis for the FDA to issue a warning letter, withdraw approvals for product candidates previously granted to us, or take other regulatory or legal action, including recall or seizure, total or partial suspension of production, suspension of ongoing clinical trials, refusal to approve pending applications or supplemental applications, detention of product, refusal to permit the import or export of products, injunction, or imposing civil and criminal penalties.

Our current manufacturer and any future manufacturers may not be able to manufacture our product candidates at a cost or in quantities or in a timely manner necessary to make commercially successful products. If we successfully commercialize any of our product candidates, we may be required to establish large-scale commercial manufacturing capabilities. In addition, as our drug development pipeline increases and matures, we will have a greater need for clinical study and commercial manufacturing capacity. We have no experience manufacturing pharmaceutical products on a commercial scale and some of these manufacturers will need to increase their scale of production to meet our projected needs for commercial manufacturing, the satisfaction of which may not be met on a timely basis.

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

We have relied upon and plan to continue to rely upon third-party contract research organizations, or CROs, and clinical data management organizations to monitor and manage data for our ongoing preclinical and clinical programs. Although we control only certain aspects of their activities, we are responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol and legal, regulatory and scientific standards, and our reliance on the CROs does not relieve us of our regulatory responsibilities. We also rely on third parties to conduct our preclinical studies in accordance with Good Laboratory Practice, or GLP, requirements and the Laboratory Animal Welfare Act of 1966 requirements. We, our CROs and our clinical trial sites are required to comply with regulations and current Good Clinical Practices, or GCP, and comparable foreign requirements to ensure that the health, safety and rights of patients are protected in clinical trials, and that data integrity is assured. Regulatory authorities ensure compliance with GCP requirements through periodic inspections of trial sponsors and trial sites. If we, any of our CROs or our clinical trial sites fail to comply with applicable GCP requirements, the clinical data generated in our clinical trials or a specific site may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications.

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

Our CROs may also fail to meet expected timelines as a result of circumstances beyond their control, including the ongoing COVID-19 pandemic. As a result of the ongoing COVID-19 pandemic, we have experienced delays in clinical trial site activation and slower patient enrollment in our clinical trials of mavorixafor in patients with WHIM and SCN, which could delay our ability to obtain regulatory approval for or successfully commercialize our product candidates.

Disruptions in our supply chain could delay the commercial launch of our product candidates.

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

Additionally, if the COVID-19 pandemic persists for an extended period of time and begins to impact essential distribution systems such as FedEx and postal delivery, we could experience disruptions to our supply chain and operations, and associated delays in the manufacturing and supply of our products, which would adversely impact our ability to deliver products to clinical trial sites or to generate sales of and revenues from our approved products.

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 are exposed to the risk that our employees, principal investigators, CROs 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, particularly in light of the work-from-home policies we have implemented in response to the ongoing COVID-19 pandemic and applicable stay-at-home orders, 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.

Future development and potential commercialization of mavorixafor in ccRCC will be pursued only as part of a potential strategic collaboration. In addition, we have entered into an agreement with Abbisko Therapeutics to develop and commercialize mavorixafor, in combination with checkpoint inhibitors or other agents in Greater China for oncology indications. No assurance can be given, however, that any current or future strategic collaboration will prove to be successful.

Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

If we decide to expand or modify one or our existing collaboration agreements or to collaborate with a new third party in connection with any of our development programs or product candidates, we may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development program or the

product candidate for which we are seeking to collaborate, reduce or delay our development program or one or more of our other development programs, delay our potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue.

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

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

Collaborations involving our product candidates pose many risks to us, including that:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborator's strategic focus or available funding or external factors such as an acquisition that diverts resources or creates competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates or products if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- collaborators with marketing and distribution rights to one or more product candidates or products may not commit sufficient resources to the marketing and distribution of such drugs;
- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our proprietary information or expose us to potential litigation;
- disputes may arise between the collaborators and us that result in the delay or termination of the research, development or commercialization of our product candidates or products or that result in costly litigation or arbitration that diverts management attention and resources;
- we may lose certain valuable rights under circumstances identified in our collaborations if we undergo a change of control;
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates; and
- collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. In addition, if a future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program under such collaboration could be delayed, diminished or terminated.

We may engage in future acquisitions or in-licenses of technology that could disrupt our business, cause dilution to our stockholders and harm our financial condition and operating results.

While we currently have no specific plans to acquire any other businesses or in-license any additional products or technology, we may, in the future, make acquisitions or licenses of, or investments in, companies, products or technologies that we believe are a strategic or commercial fit with our current product candidates and business or otherwise offer opportunities for us. In connection with these acquisitions or investments, we may:

- issue stock that would dilute our stockholders' percentage of ownership;
- expend cash;
- incur debt and assume liabilities; and
- incur amortization expenses related to intangible assets or incur large and immediate write-offs.

We also may be unable to find suitable acquisition or license candidates and we may not be able to complete acquisitions or licenses on favorable terms, if at all. If we do complete an acquisition or license, we cannot assure you that it will ultimately strengthen our competitive position or that it will not be viewed negatively by customers, financial markets or investors.

Further, the Merger poses, and future acquisitions or licenses could also pose, numerous additional risks to our operations, including:

- problems integrating the purchased or licensed business, products or technologies;
- increases to our expenses
- the failure to have discovered undisclosed liabilities of the acquired or licensed asset or company;
- diversion of management's attention from their day-to-day responsibilities;
- harm to our operating results or financial condition;
- entrance into markets in which we have limited or no prior experience; and
- potential loss of key employees, particularly those of the acquired entity.

We may not be able to complete one or more acquisitions or effectively integrate the operations, products or personnel gained through any such acquisition without a material adverse effect on our business, financial condition and results of operations.

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 recent changes to the patent laws and to the rules of the United States Patent and Trademark Office, or 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, or 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. Two cases involving diagnostic method claims and "gene patents" have recently been decided by the Supreme Court. On March 20, 2012, the Supreme Court issued a decision in Mayo Collaborative Services v. Prometheus Laboratories, Inc., or Prometheus, a case involving patent claims directed to measuring a metabolic product in a patient to optimize a drug dosage amount for the patient. According to the Supreme Court, the addition of well-understood, routine or conventional activity such as "administering" or "determining" steps was not enough to transform an otherwise patent ineligible natural phenomenon into patent eligible subject matter. On July 3, 2012, the USPTO issued guidance indicating that process claims directed to a law of nature, a natural phenomenon or an abstract idea that do not include additional elements or steps that integrate the natural principle into the claimed invention such that the natural principle is practically applied and the claim amounts to significantly more than the natural principle itself should be rejected as directed to non-statutory subject matter. On June 13, 2013, the Supreme Court issued its decision in Association for Molecular Pathology v. Myriad Genetics, Inc., or Myriad, a case involving patent claims held by Myriad Genetics, Inc. relating to the breast cancer susceptibility genes BRCA1 and BRCA2. Myriad held that isolated segments of naturally occurring DNA, such as the DNA constituting the BRCA1 and BRCA2 genes, is not patent eligible subject matter, but that complementary DNA, which is an artificial construct that may be created from RNA transcripts of genes, may be patent eligible.

We cannot assure you that our efforts to seek patent protection for our technology and products will not be negatively impacted by the changes described above, future rulings in district court cases or changes in guidance or procedures issued by the

USPTO. We cannot fully predict what impact the Supreme Court's decisions may have on the ability of life science companies to obtain or enforce patents relating to their products and technologies in the future.

Moreover, although the Supreme Court has held in Myriad that isolated segments of naturally occurring DNA are not patent-eligible subject matter, certain third parties could allege that activities that we may undertake infringe other gene-related patent claims, and we may deem it necessary to defend ourselves against these claims by asserting non-infringement and/or invalidity positions, or pay to obtain a license to these claims. In any of the foregoing or in other situations involving third-party intellectual property rights, if we are unsuccessful in defending against claims of patent infringement, we could be forced to pay damages or be subjected to an injunction that would prevent us from utilizing the patented subject matter. Such outcomes could harm our business.

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

We depend on our ability to protect our proprietary technology. We rely on trade secret, patent, copyright and trademark laws, and confidentiality, licensing and other agreements with employees and third parties, all of which offer only limited protection. Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary technology and products. Where we have the right to do so under our license agreements, we seek to protect our proprietary position by filing patent applications in the United States and abroad related to our novel technologies and products that are important to our business.

The patent positions of biotechnology and pharmaceutical companies generally are highly uncertain, involve complex legal and factual questions and have in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patents, including those patent rights licensed to us by third parties, are highly uncertain.

The steps we have taken to police and protect our proprietary rights may not be adequate to preclude misappropriation of our proprietary information or infringement of our intellectual property rights, both inside and outside the United States. The rights already granted under any of our currently issued patents and those that may be granted under future issued patents may not provide us with the proprietary protection or competitive advantages that we are seeking. If we are unable to obtain and maintain patent protection for our technology and products, or if the scope of the patent protection obtained is not sufficient, our competitors could develop and commercialize technology and products similar or superior to ours, and our ability to successfully commercialize our technology and products may be adversely affected.

With respect to patent rights, we do not know whether any of the pending patent applications for any of our product candidates will result in the issuance of patents that protect our technology or products, or which will effectively prevent others from commercializing competitive technologies and products. Our pending applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Further, the examination process may require us or our licensors to narrow the claims, which may limit the scope of patent protection that may be obtained. Although our license agreement with Genzyme includes a number of issued patents that are exclusively licensed to us, the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, issued patents that we own or have licensed from third parties may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in the loss of patent protection, the narrowing of claims in such patents, or the invalidity or unenforceability of such patents, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection for our technology and products. Protecting against the unauthorized use of our patented technology, trademarks and other intellectual property rights is expensive, difficult and may, in some cases, not be possible. In some cases, it may be difficult or impossible to detect third party infringement or misappropriation of our intellectual property rights, even in relation to issued patent claims, and proving any such infringement may be even more difficult.

We could be required to incur significant expenses to obtain our intellectual property rights, and we cannot ensure that we will obtain meaningful patent protection for our product candidates.

The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. In addition, it is also possible that we will fail to identify patentable aspects of further inventions made in the course of our development and commercialization activities before they are publicly disclosed, making it too late to obtain patent protection on them. Further, given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. We expect to seek extensions of patent terms where these are available in any countries where we are prosecuting patents. This includes in the United States under the Drug Price Competition and Patent Term Restoration Act of 1984, which permits a patent term extension of up to five years beyond the

expiration of a patent that covers an approved product where the permission for the commercial marketing or use of the product is the first permitted commercial marketing or use, and as long as the remaining term of the patent does not exceed 14 years. However, the applicable authorities, including the FDA in the United States, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or may grant more limited extensions than we request. If this occurs, our competitors may be able to take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. The laws of foreign countries may not protect our rights to the same extent as the laws of the United States, and these foreign laws may also be subject to change. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or in some cases not at all. Therefore, we cannot be certain that we or our licensors were the first to make the inventions claimed in our owned or licensed patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions.

In March 2013, the United States transitioned to a “first to file” system in which the first inventor to file a patent application will be entitled to the patent. Third parties are allowed to submit prior art prior to the issuance of a patent by the USPTO and may become involved in post-grant review or derivation proceedings for applications filed on or after March 16, 2013, interference proceedings for applications filed before March 16, 2013, ex parte reexamination, or *inter partes* review challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, which could adversely affect our competitive position with respect to third parties.

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.

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

The global coronavirus pandemic is adversely affecting, and is expected to continue to adversely affect, our business, including our clinical trials and preclinical studies.

Our business could be adversely affected by public health crises such as pandemics or similar outbreaks in regions where we have concentrations of clinical trial sites or other business operations, and could cause significant disruption in the operations of third party manufacturers and contract research organizations, or CROs, upon whom we rely. The ongoing COVID-19 pandemic has resulted in travel and other restrictions in order to reduce the spread of the disease, including state and local orders across the United States and other countries worldwide, which, among other things, have directed individuals to stay at their places of residence, direct businesses and governmental agencies to cease non-essential operations at physical locations, prohibit certain non-essential gatherings, and order cessation of non-essential travel. In March 2020, the Governor of Massachusetts ordered all individuals living in the Commonwealth of Massachusetts to stay at their place of residence for an indefinite period of time (subject to certain exceptions to facilitate authorized necessary activities) to mitigate the impact of the COVID-19 pandemic, so our workforce transitioned to working remotely. Even as certain restrictions have started to lift, we have elected to maintain remote working arrangements in the interest of minimizing risk to our employees. The effects of our work-from-home policies, as well as any executive orders and shelter-in-place or stay-at-home orders that may be implemented or re-implemented in the future, may negatively impact productivity, disrupt our business and delay our clinical programs and timelines, the magnitude of which will depend, in part, on the length and severity of the restrictions and other limitations on our ability to conduct our business in the ordinary course. In addition, because of the challenging immune profile of both immunodeficient patients and cancer patients, COVID-19 may impact our clinical trial patients more significantly than clinical trials with patients who are not immunocompromised. These and similar, and perhaps more severe, disruptions in our operations could negatively impact our business, operating results and financial condition.

Quarantines, stay-at-home and similar government orders related to COVID-19 may adversely impact our business operations and the business operations of our CROs conducting our clinical trials and our third-party manufacturing facilities in the United States and other countries. We cannot guarantee that these third parties will continue to perform their contractual duties in a timely and satisfactory manner as a result of the COVID-19 pandemic, which could negatively impact our supply chain activities and our clinical supply. We have experienced challenges in providing trial drugs to patients enrolled in our clinical trials, and where necessary and practical have implemented direct-to-patient shipments from clinical sites. If the COVID-19 pandemic persists for an extended period of time and begins to impact essential distribution systems such as FedEx and postal delivery, we could experience disruptions to our supply chain and operations, and associated delays in the manufacturing and supply of our product candidates, which would adversely impact our ability to carry out our clinical trials.

As a result of the COVID-19 outbreak, or similar pandemics, we are experiencing disruptions that could severely impact our business, clinical trials and preclinical studies, including:

- delays or difficulties in enrolling patients in our clinical trials, including travel restrictions on patients and constraints on the capacity of our clinical trial sites;
- delays or difficulties in clinical site activation, including difficulties in training clinical site investigators and clinical site staff;
- diversion or prioritization of healthcare resources away from the conduct of clinical trials and towards the COVID-19 pandemic, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials, particularly for clinical trials that require in-patient monitoring following administration of the product candidate;
- delays or disruptions in the availability of clinical site staff, who, as healthcare providers, may have heightened exposure to COVID-19, would adversely impact our clinical trial operations;
- interruption of our key clinical trial activities, such as clinical assessments at pre-specified timepoints during the trial and clinical trial site data monitoring, due to limitations on travel imposed or recommended by governmental entities, employers and others or interruption of clinical trial subject visits and study procedures (particularly any procedures that may be deemed non-essential), which may impact the integrity of subject data and clinical study endpoints;
- limitations on employee resources that would otherwise be focused on the conduct of our preclinical studies and clinical trials, including because of sickness of employees or their families, the desire of employees to avoid contact with large groups of people, an increased reliance on working from home or mass transit disruptions; and
- reduced ability to engage with the medical and investor communities due to the cancellation of conferences scheduled throughout the year.

These and other factors arising from the COVID-19 pandemic could worsen in countries that are already afflicted with COVID-19, could continue to spread to additional countries, or could return to countries where the pandemic has been partially contained, each of which could continue to adversely impact our ability to conduct clinical trials and our business generally, and could have a material adverse impact on our operations and financial condition and results.

The spread of COVID-19, which has caused a broad impact globally, may materially affect us economically. While the potential economic impact brought by, and the duration of, COVID-19 may be difficult to assess or predict, a widespread and sustained pandemic could result in significant disruption of global financial markets, reducing our ability to access capital, which could in the future negatively affect our liquidity. In addition, a recession or market correction resulting from the spread of COVID-19 could materially affect our business and the value of our common stock.

The global COVID-19 pandemic continues to rapidly evolve. The extent to which the COVID-19 pandemic will continue to impact our business, our clinical development and regulatory efforts will depend on future developments that are highly uncertain and cannot be predicted with confidence, such as the duration of the outbreak, travel restrictions, quarantines, lock-downs, social distancing requirements, business closures in the United States and other countries, and the effectiveness of actions taken in the United States and other countries to contain and treat the disease. Accordingly, we do not yet know the full extent of potential delays or impacts on our business, our clinical and regulatory activities, healthcare systems or the global economy as a whole.

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 highly dependent upon members of our current management team, including Paula Ragan, Ph.D., our Chief Executive Officer, the loss of whose services may adversely impact the achievement of our objectives. Although we have an employment agreement with Dr. Ragan, this agreement is at-will and does not prevent her from terminating her employment with us at any time by providing the requisite advance notice.

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, 2020, we had 68 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. Because of the work-from-home policies we implemented due to COVID-19, information that is normally protected, including company confidential information, may be less secure. 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, due to the COVID-19 pandemic, we have enabled substantially all of our employees to 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 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.

Business disruptions could seriously harm our future revenues and financial condition and increase our costs and expenses.

Our operations could be subject to earthquakes, power shortages, telecommunications failures, water shortages, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics (including but not limited to the COVID-19 pandemic) and other natural or man-made disasters or business interruptions, for which we are predominantly self-insured. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses. We rely on a single third-party manufacturer to provide the active pharmaceutical ingredient for mavorixafor and a single third-party manufacturer to provide fill and finish services for the final drug product formulation of mavorixafor for use in clinical trials. Our ability to obtain clinical supplies of product candidates could be disrupted if the operations of these suppliers are affected by a man-made or natural disaster or other business interruption.

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

Our net operating loss, or 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, 2019, we had U.S. federal and state NOLs of $181.8 million and $177.4 million, respectively. Our NOLs generated in tax years ending on or prior to December 31, 2017 are only permitted to be carried forward for 20 years under applicable U.S. tax law. Under the Tax Act, as modified by the CARES Act, our federal NOLs generated in tax years ending after December 31, 2017 may be carried forward indefinitely, but the deductibility of federal NOLs, particularly for tax years beginning after December 31, 2020, may be limited. It is uncertain if and to what extent various states will conform to the Tax Act and the CARES Act.

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

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

In October 2018, we entered into a loan and security agreement, as most recently amended in March 2020, with Hercules, secured by a lien on substantially all of our assets, including 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, to the extent borrowings on the loan and security agreement exceed $25.0 million, maintaining a minimum liquidity amount of the lesser of (i) $30.0 million or (ii) 6 multiplied by a metric based on prior months' cash expenditures; provided, however, that from and after the Company's achievement of certain performance milestones, the required level shall be reduced to the greater of (x) $20.0 million, or (y) 3 multiplied by the current metric based on prior months' cash expenditures. 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.

Changes in U.S. tax law may materially adversely affect our financial condition, results of operations and cash flows.

On March 27, 2020, the CARES Act was signed into law. The CARES Act is an approximately $2 trillion emergency economic stimulus package that includes numerous U.S. federal income tax provisions, including the modification of: (i) net operating loss (NOL) rules (as discussed above), (ii) the alternative minimum tax refund and (iii) business interest deduction limitations under Section 163(j) of the Internal Revenue Code of 1986, as amended (the Code).

The Tax Act also significantly changed the U.S. federal income taxation of U.S. corporations. However, there remain uncertainties and ambiguities in the application of certain provisions of the Tax Act and, as a result, we made certain judgments and assumptions in the interpretation thereof. The U.S. Treasury Department and the Internal Revenue Service, or the IRS, may issue further guidance on how the provisions of the Tax Act will be applied or otherwise administered that differs from our current interpretation. In addition, the Tax Act could be subject to potential amendments and technical corrections, any of which could materially lessen or increase certain adverse impacts of the legislation on us. We continue to work with our tax advisors and auditors to determine the full impact the Tax Act and the CARES Act will have on us. We urge our investors to consult with their legal and tax advisors with respect to both the Tax Act and the CARES Act and the potential tax consequences of investing in our common stock.

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 Ownership of Our Common Stock

Our stock price is expected to continue to be volatile.

The market price of our common stock could continue to be subject to significant fluctuations. Market prices for securities of early-stage 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 market or macroeconomic conditions;
- 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
- general economic, industry, political and market conditions, including, but not limited to the ongoing impact of the COVID-19 pandemic.

In addition, companies trading in the stock market in general, and Nasdaq in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies, including very recently in connection with the ongoing 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 ongoing 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.

We will be required to raise additional funds to finance our operations; we may not be able to do so when necessary, and/or on acceptable terms.

Our ongoing capital requirements will depend on numerous factors related to the development of our product candidates and the sale of products obtaining regulatory approval, including: the progress and cost of research and development programs and clinical trials; the progress and cost of research and development programs of collaborators; the time and costs expended and required to obtain any necessary or desired regulatory approvals; the costs of ongoing compliance with the FDA and other domestic and foreign regulatory agency requirements; the resources devoted to manufacturing expenditures; the ability to enter into licensing arrangements; the cost of commercialization activities and arrangements, if any, undertaken by us; and, if and when approved, the demand for our products.

We anticipate that we will need to raise additional funds through public or private financings, strategic collaborations or other arrangements. The spread of COVID-19, which has caused a broad impact globally, may materially affect our ability to access the capital markets when and if needed. While the long-term economic impact of the COVID-19 pandemic is difficult to assess or predict, it has already significantly disrupted global financial markets, reducing our ability to access capital, which could in the future negatively affect our liquidity. Additional equity financing would be dilutive to our existing stockholders, and debt financing, if available, may involve restrictive covenants. If we raise funds through collaborative or licensing arrangements, we may be required to relinquish, on terms that are not favorable to us, rights to some of our technologies or product candidates that we would otherwise seek to develop or commercialize. Our failure to raise capital when needed could materially harm our business, financial condition and results of operations.

Raising additional funds by issuing securities may cause dilution to existing stockholders and raising funds through lending and licensing arrangements may restrict our operations or require us to relinquish proprietary rights.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, grants and license and development agreements in connection with any collaborations. We do not have any committed external source of funds. To the extent that we raise additional capital by issuing equity securities, our existing stockholders' ownership will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing and preferred equity 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 or declaring dividends.

If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. Any debt financing that we enter into may involve covenants that restrict our operations. These restrictive covenants may include limitations on additional borrowing and specific restrictions on the use of our assets as well as prohibitions on our ability to create liens, pay dividends, redeem our stock or make investments. If we are unable to raise additional funds through equity or debt financings 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 are an "emerging growth company," and a "smaller reporting company" and as a result of the reduced disclosure requirements applicable to emerging growth companies and smaller reporting companies, our common stock may be less attractive to investors.

We are an "emerging growth company," or EGC, as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and may remain an emerging growth company until December 31, 2022. For so long as we remain an EGC, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include:

- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding a supplement to the auditor's report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation; and
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and shareholder approval of any golden parachute payments not previously approved.

Investors may find our common stock less attractive as a result of our reliance on these exemptions. If some investors find our common stock 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.

Even following the termination of our status as an emerging growth company, we will be able to take advantage of the reduced disclosure requirements applicable to smaller reporting companies (as that term is defined in Rule 12b-2 of the Exchange Act) and, in particular, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements. To the extent that we are no longer eligible to use exemptions from various reporting requirements, we may be unable to realize our anticipated cost savings from these exemptions, which could have a material adverse impact on our operating results.

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.

Additionally, a holder of our common stock has rights, subject to some conditions, to require us to file one or more registration statements covering their shares. These registration rights will terminate upon the earlier to occur of November 17, 2020 or the date on which the holder has sold all shares subject to such registration rights. If we were to register the resale of these shares, they could be freely sold in the public market. If these additional shares are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

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 Exchange Act, the Sarbanes-Oxley Act of 2002 and the rules and regulations of The Nasdaq Global Market. Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or 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 Form 10-K.

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 of the Sarbanes-Oxley Act, or 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 our Annual Report on Form 10-K for the year ended December 31, 2019. However, while we remain an EGC, 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 an emerging growth company, 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 The Nasdaq Global Market, the SEC or other regulatory authorities.

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, including litigation, if any, that may result in connection with the Merger. Litigation often is expensive and diverts management's attention and resources, which could adversely affect our business.

Provisions in our corporate charter documents and under Delaware law could make an acquisition of the 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 the 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

None.

Item 6. EXHIBITS

Exhibit No.	Exhibit Description	Incorporated by Reference to:			
		Form	Exhibit No.	Filing Date	File No.
3.1	Restated Certificate of Incorporation, as amended, as of June 10, 2020.	8-K	3.1	06/10/2020	001-38295
3.2	Amended and Restated By-laws of the Company	8-K	3.2	11/20/2017	001-38295
10.1	Controlled Equity Offering℠ Sales Agreement, dated August 7, 2020, by and among the Registrant and B. Riley Securities, Inc., Cantor Fitzgerald & Co. and Stifel, Nicolaus & Company, Incorporated.	S-3	1.2	08/07/2020	333-242372
10.2*+	Consulting agreement, dated September 17 2020, by and between X4 Pharmaceuticals Inc. and Gary Bridger				
10.3*	Lease Termination Agreement, dated July 17, 2020, by and between 955 Massachusetts Avenue MA, LLC and X4 Pharmaceuticals, Inc.				
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)

* 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.
+ Indicates management contract or compensatory plan

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 5, 2020

By: /s/ Paula Ragan, Ph.D.
Paula Ragan, Ph.D.
President, Chief Executive Officer and Secretary

Date: November 5, 2020

By: /s/ Adam S. Mostafa
Adam S. Mostafa
Chief Financial Officer and Treasurer

September 17, 2020

Gary Bridger, Ph.D.
445 South Garden Street
Bellingham, WA 98225

Re: Independent Contractor Agreement

Dear Dr. Bridger:

We are pleased that you ("**Consultant**") have agreed to provide consulting services to X4 Pharmaceuticals, Inc. (the "**Company**"). This letter is being written for the purpose of setting forth the basic terms of the understandings between you and the Company. If you are in agreement with these terms, please sign and date the last page of one copy of this letter and return it to us, whereupon this letter shall represent a legally binding agreement (the "**Agreement**"). Please sign and keep the other copy of this letter for your files.

In consideration of the mutual promises and covenants contained in this Agreement, and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, we have agreed as follows:

1. Services. Consultant agrees to personally render to the Company the services described on Schedule A attached hereto and incorporated herein by reference, as well as such other duties which may be requested by the Company from time to time and which are necessary and reasonably related to the successful completion of the services described in Schedule A attached hereto (collectively, the "**Services**").

2. Term of Consulting Arrangement. This Agreement shall be effective as of September 17, 2020 (the "**Effective Date**") and shall expire on December 17, 2020, unless mutually extended by the parties, or earlier terminated by either party (the "**Term**") as provided below. Expiration or termination of this Agreement shall not affect accrued rights or obligations of the parties. Notwithstanding the foregoing, either the Company or Consultant may terminate this Agreement at any time, effective immediately, for a material breach which remains uncured for thirty (30) days after written notice thereof is given to the defaulting party. In addition, either party may terminate this Agreement at any time and without cause by providing at least thirty (30) days prior written notice of such termination to the other party. The provisions of Paragraphs 4, 5, 6, 7, 8, 10(c), and 10(d) of this Agreement will survive any termination of this Agreement.

3. Consideration. In consideration of Consultant's performance of the Services, the Company shall provide Consultant with the following during the Term:

a. **Compensation**: In exchange for Consultant's Services, the Company shall pay Consultant a fee of thirty thousand dollars ($30,000.00) per month. Consultant shall submit an invoice to the Company, via e-mail to accounts.payable@x4phama.com on a monthly basis. **Such invoices shall reference X4 PO# X4C20-0301.** Absent a good faith dispute, the Company shall provide payment to Consultant for such Services within thirty (30) business days following

receipt of such invoices. The payments provided for in this Section 3 will constitute Consultant's sole compensation for all Services rendered to the Company hereunder and all benefits conferred upon the Company hereunder. No additional payments shall be due hereunder unless specifically agreed to in writing by the Company and Consultant.

b. **Option Grant**: Subject to approval by the Company's Board of Directors of the Company (the "**Board**"), the Company will grant Consultant a non-qualified stock option (the "**Option**") for the purchase of an aggregate of 30,000 shares of Common Stock of the Company at a price per share equal to the fair market value on the date of grant, under the Company's 2017 Equity Incentive Plan (the "**Plan**"). The Option shall be subject to all terms, vesting, and other provisions set forth in a separate option agreement under the Plan. The Option will have a term of 10 years, except as set forth in the option agreement and the Plan, and be subject to a vesting schedule of three (3) years, with 33% of the shares vesting on the first anniversary after the vesting commencement date, and the remainder vesting in equal installments over the following 24 months, provided that Consultant remains a Director on the Board on such vesting dates.

c. **Expense Reimbursement**: The Company will reimburse Consultant for reasonable out-of-pocket business and travel expenses incurred by Consultant in performing the Services (including lodging expenses and (i) coach class airfare or (ii) business class or better airfare for travel of more than six (6) hours or if traveling between the US and a foreign country and Acela Express if travel is by train). Consultant shall record such approved expenses on the invoice described in paragraph (a) above, and, absent a good faith dispute, the Company shall provide payment to Consultant for such expenses within thirty (30) business days following receipt of such invoices. Consultant must submit a prior written request to incur any such expenses in excess of two hundred dollars ($200), and may not incur such expense without the Company's prior written approval. The Company shall have no obligation to reimburse Consultant for unreasonable expenses, expenses that are not related to Company business, and/or expenses in excess of the threshold amount described above that were not approved in advance by the Company.

d. **General:** In the event this Agreement is terminated by Consultant or by the Company for any reason, no compensation of any kind shall be payable or issuable to Consultant after the effective date of such termination, other than a liability or obligation of either party which accrued prior to such termination including but not limited to any business expenses that are not cancellable.

4. **Confidential Information.** Consultant recognizes and acknowledges that the Company's trade secrets, know-how and proprietary processes as they exist from time to time as well as other confidential and proprietary information, including the Company's confidential business plans, preclinical and clinical data, operations and procedures, manufacturing methods and techniques, processes, formulas, designs, products, regulatory status and strategies, technical infrastructure, financial information (hereinafter, "**Confidential Information**") are and shall be the exclusive property of the Company. Consultant shall hold all Confidential Information in strict confidence. Further, Consultant shall not, during and after the term of consultancy to the

Company, in whole or in part, disclose such Confidential Information to any person, firm, corporation, association or other entity for any reason or purposes whatsoever, nor shall Consultant make use of any property owned by the Company for Consultant's own purposes or for the benefit of any third party (except the Company) under any circumstances during or after the term of the consultancy. These restrictions shall not apply to such secrets, know-how and processes which Consultant can establish by written proof:

i. were known to Consultant, other than under binder of secrecy, prior to consulting to the Company;

ii. have passed into the public domain prior to or after their development by or for the Company, or their disclosure to the Company, other than through acts or omissions attributable to Consultant; or

iii. were subsequently obtained, other than under binder of secrecy, from a third party not acquiring the information under an obligation of confidentiality from the disclosing party.

The Company has taken and shall continue to take all reasonable measures to protect the confidentiality of Confidential Information because of its great value to the Company. Upon termination of this Agreement for any reason, Consultant shall promptly return to the Company all records, files, memoranda, notes, designs, data, reports, price lists, customer lists, drawings, plans, computer programs, software, software documentation, sketches, laboratory and research notebooks and other documents (and all copies or reproductions of such materials) containing or relating to Confidential Information. Consultant will not disclose to the Company any confidential information, proprietary material or trade secrets belonging to any current or former employer or other third party.

Consultant acknowledges that certain of the foregoing types of Confidential Information disclosed to Consultant, or of which Consultant otherwise becomes aware of, during the term of this Agreement may be or be deemed to be "material non-public information" ("MNPI") within the meaning of the federal or state securities laws. MNPI will only be disclosed to Consultant on a need to know basis and for the specific purpose of providing the Services, and Consultant shall use such MNPI only for such specific purpose. Consultant (on behalf of itself and any of its affiliates) agrees to abide by all securities and related laws, rules and regulations in connection with providing the Services including, without limitation, those laws, rules and regulations relating to the receipt, handling and use of MNPI. If requested by the Company, Consultant agrees to execute and comply with the Company's Insider Trading policy applicable to its directors and employees.

Consultant (on behalf of itself and its affiliates) agrees that it will not buy or sell common stock or other securities (including, but not limited to, derivatives and options) of X4 Pharmaceuticals, Inc. on the basis of MNPI regarding X4 Pharmaceuticals or otherwise. During the term of the Agreement and for a minimum of three (3) years thereafter, Consultant for itself and its agents agrees not to sell X4 Pharmaceuticals' common stock on a "short" basis.

5. **Ownership of Intellectual Property.** Consultant hereby transfers and assigns to the Company, or to any person or entity designated by the Company, Consultant's entire right, title, and interest in and to all inventions, ideas, discoveries and improvements (including, but not limited to, information regarding small molecule antagonists, targets, cell lines, cell culture, manufacturing methods, animal models, assay procedures, clinical development plans, and clinical trial protocols), whether patented or unpatented, and material subject to copyright, made or conceived by Consultant, solely or jointly, arising out of the performance of the Services, whether or not conducted at the Company's facilities (all of which are collectively referred to herein as "**Inventions**"). Notwithstanding anything to the contrary, Inventions shall not include any Consultant Property defined to mean all ideas, concepts, know-how, techniques and processes of a general nature that are discovered, invented, created, conceived, made or reduced to practice by Consultant (i) prior to performing Services; or (ii) upon or after performance of the Services, provided that (A) such Consultant Property was developed independent of the scope of Services specifically for Company; (B) such Consultant Property is not based on or derived, in whole or in part, directly or indirectly, from Company data or Company's intellectual property and (C) Consultant shall retain all right, title and interest in and to the Consultant Property. The Company shall own and have title to any Inventions made during the Term of this Agreement. Consultant shall communicate promptly and disclose to the Company, in such form as the Company may reasonably request, all information, details and data pertaining to any Inventions; and Consultant shall execute and deliver to the Company such formal transfers and assignments and such other papers and documents and at Company's expense for Consultant's time shall give such testimony as may be necessary or required of Consultant to permit the Company to file and prosecute patent applications and, as to material subject to copyright, to obtain copyrights thereof. Consultant acknowledges that all copyrightable materials developed or produced by Consultant during the performance of the Services constitute "works made for hire" pursuant to the United States Copyright Act (17 U.S.C. Section 101). In the event that Consultant is requested in any proceeding to disclose any Confidential Information, Consultant shall give the Company prompt notice of such request so that the Company may seek an appropriate protective order. If, in the absence of a protective order, Consultant is nonetheless compelled by order or subpoena of any court or tribunal of competent jurisdiction to disclose Confidential Information, Consultant may disclose such information without liability hereunder; provided, that, Consultant shall give the Company notice of the Confidential Information to be disclosed as far in advance of its disclosure as is practicable and use Consultant's reasonable best efforts to obtain assurances that confidential treatment will be accorded to such Confidential Information.

6. **No Limitations with Other Entities.** Consultant shall be free to provide professional consulting services to entities or individuals other than the Company so long as Consultant meets Consultant's minimum monthly service obligations to the Company as described in Schedule A attached hereto. Consultant shall provide to the Company prior written notice of Consultant's intention to render professional consulting services to any entity or individual which provides services that are in direct competition with the Services rendered by Consultant hereunder, including with respect to CXCR4-targeted therapies.

7. **Consultant Documents; Equipment.** Immediately upon the Company's request and promptly upon termination of this Agreement, Consultant shall deliver to the Company all

memoranda, notes, records, reports, photographs, drawings, plans, papers or other documents made or compiled by Consultant or made available to Consultant during the term of this Agreement, and copies or abstracts thereof, whether or not of a secret or confidential nature (collectively, the "**Consultant Documents**") as well as any equipment provided to Consultant by the Company or at Company's expense ("**Equipment**"). Both Consultant Documents and Equipment shall be and are the exclusive property of the Company to be used by Consultant only in the performance of its duties for the Company.

8. Indemnification and Insurance. Consultant shall defend, indemnify and hold harmless the Company, its employees, officers and agents from and against any and all claims, demands, loss, damage or expense, including without limitation attorneys' fees (herein, "**Claims**"), that arise as a result of Consultant's performance of or failure to perform the Services and breach of the terms of this Agreement. Company shall defend, indemnify and hold harmless the Consultant, its employees, officers and agents from and against any and all Claims, that arise as a result of Company's use of the Services and breach of the terms of this Agreement.

9. Independent Contractor Status; No Employment Created. Consultant acknowledges that the relationship of Consultant to the Company is at all times that of an independent contractor. This Agreement does not constitute, and shall not be construed as constituting, an employment relationship between the Company and any persons or as an undertaking by the Company to hire Consultant or any persona as an employee of the Company. The Company will not provide Consultant with an office or any other space from which to conduct the Services, and Consultant shall have the sole control and discretion as to where to perform the Services. Consultant will perform the Services free of the direction and control of the Company, but consistent with the objectives it sets, and will bear the benefit/risk of any profit or loss from rendering the Services. Consultant will not be considered an employee of the Company for any purpose, including without limitation, any Company employment policy or any employment benefit plan, and will not be entitled to any benefits under any such policy or benefit plan (including without limitation Workers Compensation insurance). Consultant shall obtain workers' compensation insurance for Consultant and shall provide the Company with evidence of such coverage satisfactory to it. The Company will record payments to Consultant on an Internal Revenue Service Form 1099, and will not withhold any federal, state or local employment taxes on Consultant's behalf. Consultant will be solely responsible for the payment of all federal, state and local taxes and contributions imposed or required on income, and for all unemployment insurance, social security contributions and any other payment.

10. Miscellaneous.

a) Representations and Warranties. Except as set forth in Schedule B attached hereto, Consultant hereby represents and warrants that (i) Consultant has no commitments or obligations inconsistent with this Agreement; (ii) Consultant will perform the Services in compliance with all applicable laws, rules, and regulations, (iii) Consultant will perform the Services to the best of Consultant's ability in a professional manner consistent with academic, scientific and/or industry standards, in accordance with the standard of care customarily observed with regard to such Services in Consultant's profession and using the Consultant's expertise, and

(iv) the performance by Consultant of the Services do not as of the Effective Date and shall not at any time during the Term conflict with, breach any covenants or agreements under which Consultant is performing services or research for Consultant's current employer or other client(s), if applicable. Consultant hereby agrees to indemnify and hold the Company harmless against any claim based upon circumstances alleged to be inconsistent with such representations and/or warranties.

b) Notices. Any notice or other communication required or permitted hereunder shall be deemed sufficiently given if sent by facsimile transmission, recognized courier service or certified mail, postage and fees prepaid, addressed to the party to be notified as follows: if to the Company to its address set forth above, and if to Consultant to Consultant's address set forth above, or in each case to such other address as either party may from time to time designate in writing to the other. Such notice or communication shall be deemed to have been given as of the date sent by facsimile or delivered to a recognized courier service, or three days following the date deposited with the United States Postal Service.

c) Governing Law; Arbitration. This Agreement shall be governed by and construed and enforced in accordance with the laws of the Commonwealth of Massachusetts (including without limitation its laws regarding trade secrets), without application of the conflicts of law provisions thereof. Except with respect to the provisions of Sections 4, 5 and 7 hereof (for which the Company expressly reserves the right to seek injunctive relief in the event of a breach or threatened breach, as described in paragraph (d) below), any controversy, dispute or claim arising out of or in connection with this Agreement, or the breach, termination or validity hereof, shall be settled by final and binding arbitration to be conducted by a single arbitrator in Boston, Massachusetts, pursuant to the Commercial Rules of the American Arbitration Association. The decision or award of the arbitrator shall be final, and judgment upon such decision or award may be entered in any competent court or application may be made to any competent court for judicial acceptance of such decision or award and an order of enforcement. In the event of any procedural matter not covered by the aforesaid rules, the procedural law of the Commonwealth of Massachusetts shall govern.

d) Injunctive Relief; Severability; Blue Pencil. Consultant hereby expressly acknowledges that any breach or threatened breach of any of the terms and/or conditions set forth in Sections 4, 5 and 7 of this Agreement may result in substantial, continuing and irreparable injury to the Company. Therefore, Consultant hereby agrees that, in addition to any other remedy that may be available to the Company, the Company shall be entitled to seek injunctive or other equitable relief by a court of appropriate jurisdiction in the event of any breach or threatened breach of the terms of Sections 4, 5 or 7 of this Agreement. In addition, the parties intend Sections 4, 5 and 7 of this Agreement to be enforced as written. However, (i) if any provision of such sections is to any extent declared illegal or unenforceable by a duly authorized court having jurisdiction, then the remainder of such sections, or the application of such provision in circumstances other than those as to which it is so declared illegal or unenforceable, will not be affected thereby, and each provision of such sections will be valid and enforceable to the fullest extent permitted by law; and (ii) if any provision of such sections, or part thereof, is held to be unenforceable because of the duration of such provision or the geographic area covered thereby, the court making such determination will have the power to reduce the duration and/or geographic area of such provision, and/or to delete specific words and phrases ("blue-

penciling"), and in its reduced or blue-penciled form such provision will then be enforceable and will be enforced.

e) Entire Agreement. This Agreement embodies the entire agreement and understanding between the parties hereto and supersedes all prior oral or written agreements and understandings relating to the subject matter hereof, including, but not limited to, the Independent Contractor Agreement between Consultant and Company, entered into on or about November 14, 2017 and all amendments thereto. No statement, representation, warranty, covenant or agreement of any kind not set forth in this Agreement shall affect, or be used to interpret, change or restrict, the express terms and provisions of this Agreement.

f) Assignment. The rights and obligations of Consultant and the Company hereunder shall inure to the benefit of, and shall be binding upon, their respective successors and assigns; provided, however, that nothing contained in this Agreement shall restrict or limit the Company, in any manner whatsoever, from assigning any or all of its rights, benefits or obligations under this Agreement to any affiliate of the Company or in connection with a merger, acquisition or other corporate transaction, in either case, without the necessity of obtaining Consultant's consent. Consultant's rights and obligations under this Agreement may not be assigned without the prior written consent of the Company.

g) Modification and Amendment. This Agreement shall not be modified, amended or extended except by an instrument in writing signed by or on behalf of the parties hereto.

h) Counterparts. This Agreement may be executed in one or more counterparts each of which will be deemed an original, but all of which together shall constitute one and the same instrument. Signatures to this Agreement transmitted by facsimile transmission, by electronic mail in "portable document format" (".pdf") form, or by any other electronic means intended to preserve the originals graphic and pictorial appearance of a document, will have the same effect as physical delivery of the paper document bearing the original signatures, and shall be deemed original signatures by both parties.

i) Interpretation. The parties hereto acknowledge and agree that (i) the rule of construction to the effect that any ambiguities are resolved against the drafting party, and (ii) the terms and provisions of this Agreement, shall be construed fairly as to all parties hereto and not in favor of or against a party, regardless of which party was generally responsible for the preparation of this Agreement.

If the foregoing accurately sets forth our agreement, please so indicate by signing and returning to us the enclosed copy of this letter.

Very truly yours,

X4 Pharmaceuticals, Inc.

/s/ Paul Ragan

Title: CEO

Name: Paula Ragan
Date:

Agreed to and Accepted:

GARY BRIDGER, Ph.D.

/s/ Gary Bridger

Date:

Schedule A

Description of Services

- For approximately 48 hours per month, Consultant will provide periodic review and guidance relating to scientific data and planning, stemming from the Company's discovery, non-clinical, clinical and translational research efforts.

Schedule B

Existing Conflicts

LEASE TERMINATION AGREEMENT

This **LEASE TERMINATION AGREEMENT** (the "**Agreement**") dated this 17th day of July 2020 (the "**Effective Date**") is made by and among **955 MASSACHSUETTS AVENUE MA, LLC**, a Delaware limited liability company**,** successor-in-interest to BRICKMAN 955 MASSACHUSETTS LLC (the "**Landlord**"), and **X4 PHARMACEUTICALS, INC.**, a Delaware corporation (the "**Tenant**").

RECITALS:

A. WHEREAS, Landlord and Tenant entered into that Lease dated January 20, 2017 (the "**Lease**") pursuant to which Tenant leases certain premises from Landlord consisting of approximately 12,577 rentable square feet (the "**Premises**") located at 955 Massachusetts Avenue, Cambridge, Massachusetts;
B. WHEREAS, the Term of the Lease is scheduled to expire on July 31, 2022 ("**Expiration Date**"); and
C. WHEREAS, Tenant and Landlord desire to terminate the Lease prior to the Expiration Date subject to the terms and conditions set forth herein.

AGREEMENT:

NOW THEREFORE, for valuable consideration, the receipt and sufficiency of which are hereby acknowledged, Landlord and Tenant hereby agree as follows:

1. Definitions. Capitalized terms not otherwise defined herein shall have the meaning assigned to them in the Lease. In the event of any conflict between the terms of the Lease and the terms of this Amendment, the definitions set forth in this Agreement shall supersede and control.
2. Incorporation of Recitals. The recitals set forth above are incorporated herein and made part of this Agreement to the same extent as if set forth herein in full.
3. Termination. Provided that Tenant delivers the Termination Payment to Landlord on the Effective Date and subject to all other terms and conditions set forth herein, Landlord and Tenant hereby agree that the Term of the Lease shall be deemed cancelled and terminated and shall be of no further force and effective as of September 30, 2020 (the "**Termination Date**").
4. Termination of Tenant's Rights & Surrender Obligations. Upon the Termination Date, Tenant hereby agrees that it shall have no further right to use, occupy, enter or otherwise enjoy the Premises and Tenant shall deliver any and all keys to the Premises to Landlord. In addition, upon the Termination Date, Tenant shall vacate and surrender the Premises to Landlord in accordance with its obligations under **Section 12.2 Surrender of Premises** of the Lease.
5. Letter of Credit. Subject to the terms and conditions set forth herein, the Letter of Credit in the amount of $264,117.00 shall be terminated by Landlord within thirty (30) days of the Effective Date.
6. Termination Fee. Tenant shall pay to Landlord the sum of $345,867.60 (the "**Termination Fee**") on the Effective Date (the "**Payment Date**") by "bank

check" or wire transfer. Notwithstanding the foregoing, in the event that Tenant fails to pay the Termination Fee to Landlord within five (5) business days of the Payment Date, this Agreement shall be automatically null and void in all respects and the Lease shall continue in full force and effect.

7. Release by Tenant. In consideration of the terms and conditions herein, Tenant shall and does hereby fully, irrevocably, finally, and unconditionally release and forever discharge Landlord, and its partners, predecessors, successors, assigns, parents, subsidiaries, and affiliates, and the respective officers, directors, employees, consultants, lawyers, agents, and representatives of the same, from any and all actions (including, without limitation, informal proceedings), debts, liens, obligations, liabilities, claims, rights, demands, damages (including, without limitation, punitive and consequential damages), judgments, losses, costs, and expenses of every kind and nature whatsoever, known or unknown, suspected or unsuspected, fixed or contingent, arising from, incidental to, based upon, or related to the Lease, whether arising before or after the Termination Date.
8. Tenant's Representations. Tenant hereby represents and warrants to Landlord as follows:
    a. Tenant has not entered into any written or oral agreements with any third parties that: (i) pertain to the provision of goods and/or services to or for the Premises that will continue in effect after the Termination Date; or (ii) either the Premises or Landlord will become subject to or liable for payment.
    b. Tenant has the full right, legal power and actual authority to enter into this Agreement and undertake the obligations hereunder.
    c. Tenant is not obligated to obtain the consent of any third party in order for this Agreement and/or the termination of the Lease to be valid, binding and effective upon all parties.
    d. Tenant is the sole owner of the Tenant's interest in the Lease, and Tenant has not made any assignment, transfer, conveyance, hypothecation, or other disposition of the Lease, or any interest therein, and Tenant has not entered into any sublease or assignment of the Premises or any portion thereof which remains in effect.
    e. Tenant: (i) has not made a general assignment for the benefit of creditors;

        (ii) has not filed any voluntary petition in bankruptcy or suffered the filing of any involuntary petition by Tenant's creditors; (iii) has not suffered the appointment of a receiver to take possession of all, or substantially all, of Tenant's assets; (iv) has not suffered the attachment or other judicial seizure of all, or substantially all, of Tenant's assets; (v) has not admitted in writing its inability to pay its debts as they come due; (vi) has not made an offer of settlement, extension or composition to its creditors generally; and (vii) is not contemplating the filing of bankruptcy.

9. Holdover. Tenant hereby acknowledges that Landlord is negotiating a new lease with respect to the Premises that commences on October 1, 2020 so it is critical that Tenant vacates the Premises in a timely manner as set forth herein. Notwithstanding the terms and conditions of the Lease to the contrary, in the event that Tenant does not surrender and vacate the Premises by the Termination Date in accordance with the terms and conditions of Section 4 herein, such

holding over shall be treated as a daily tenancy at sufferance and Tenant shall be liable to Landlord for: (a) Basic Rent and Additional Rent equal to two hundred percent (200%) of the rate in effect for the prior rental period; plus (b) any consequential damages incurred by Landlord.

10. Confidentiality. Landlord and Tenant hereby agreed to keep the terms of this Agreement strictly confidential and shall not disclose the terms of this Agreement except to those persons who are in a "need-to-know" capacity. In the event of a violation of this provision, Landlord and Tenant shall have all rights and remedies under law or at equity.

11. Contingency. This Agreement is subject to Landlord entering into a lease amendment with respect to the Premises. In the event that this condition does not occur, this Agreement shall be null and void in all respects.

12, Multiple Counterparts. This Agreement may be executed in several counterparts, each of which shall be an original and all of which shall constitute but one and the same instrument, except that in the event of variation or discrepancy between counterparts, the counterpart held by Landlord shall control.

[REMAINDER OF PAGE INTENTIONALLY LEFT BLANK] [SIGNATURE PAGE TO FOLLOW]

IN WITNESS WHEREOF, the parties hereto have executed this Agreement as of the Effective Date:

LANDLORD:
955 MASSACHUSETTS AVENUE MA, LLC
a Delaware limited liability company

By: **BAY STATE REIT, LLC**
a Delaware limited liability company, its manager

By: **U.S. REAL ESTATE INVESTMENT FUND REIT, INC.**
a Delaware corporation, its manager

By: /s/ Thomas Tarant Name: Thomas Taran
Title: Vice President

TENANT:

X4 PHARMACEUTICALS, INC.

By: /s/ Adam Mostafa
Name: Adam Mostafa
Title: CFO

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 5, 2020

/s/ Paula Ragan, Ph.D.

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

Exhibit 31.2

CERTIFICATIONS

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 5, 2020

/s/ Adam S. Mostafa

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

Exhibit 32.1

CERTIFICATIONS UNDER SECTION 906

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, 2020, to which this Certification is attached as Exhibit 32.1 (the "Periodic Report") fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, and
2. The information contained in the Periodic 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 5th day of November 2020.

Dated: November 5, 2020

/s/ Paula Ragan, Ph.D.
Name: Paula Ragan, Ph.D.
President, Chief Executive Officer and Secretary
(Principal Executive Officer)

Dated: November 5, 2020

/s/ Adam S. Mostafa
Name: Adam S. Mostafa
Chief Financial Officer and Treasurer
(Principal Financial Officer)

This certification accompanies the Form 10-Q to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of X4 Pharmaceuticals, Inc. 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 the Form 10-Q), irrespective of any general incorporation language contained in such filing.