UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): April 11, 2019

X4 Pharmaceuticals, Inc.
(Exact name of registrant as specified in its charter)

Delaware	001-38295	27-3181608
(State or other jurisdiction of incorporation)	(Commission File Number)	(IRS Employer Identification No.)

955 Massachusetts Avenue, 4th Floor Cambridge, Massachusetts	02139
(Address of principal executive offices)	(Zip Code)

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

Not applicable
(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

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. ☒

Item 8.01 Other Events.

As previously disclosed, on March 8, 2019, X4 Pharmaceuticals, Inc., formerly Arsanis, Inc. (the "Company" or "Arsanis"), Artemis AC Corp., a wholly owned subsidiary of Arsanis, X4 Therapeutics, Inc., formerly X4 Pharmaceuticals, Inc., and Arsanis Biosciences GmbH ("Arsanis GmbH"), a wholly owned subsidiary of Arsanis, entered into a settlement agreement (the "Settlement Agreement") with Österreichische Forschungsförderungsgesellschaft GmbH ("FFG"), in respect of the previously reported allegations by FFG that Arsanis and Arsanis GmbH breached certain reporting, performance and other obligations in connection with the grants and loans made by FFG to Arsanis GmbH to fund qualifying research and development expenditures. A copy of the Settlement Agreement is attached hereto as Exhibit 10.1 and incorporated by reference in this Item 8.01.

The Company has attached hereto as Exhibit 99.1 and Exhibit 99.2 and incorporated by reference in this Item 8.01 an updated description of the Company's business and updated risk factors of the Company, respectively.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit Number	Description
10.1	Settlement Agreement, dated as of March 8, 2019, by and among X4 Pharmaceuticals, Inc. (formerly Arsanis, Inc.), Artemis AC Corp., X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals, Inc.), Arsanis Biosciences GmbH and Österreichische Forschungsförderungsgesellschaft GmbH.
99.1	Description of the Business of X4 Pharmaceuticals, Inc.
99.2	Risk Factors of X4 Pharmaceuticals, Inc.

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 hereunto duly authorized.

X4 PHARMACEUTICALS, INC.

Date: April 11, 2019

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

Exhibit 10.1

Exemption from stamp duties according to Sec. 14(2) of the Act on the Establishment of the Austrian Research Promotion Agency (Forschungsförderungsgesellschaftsgesetz).

SETTLEMENT AGREEMENT

concluded between

ARSANIS Biosciences GmbH

Arsanis, Inc.

Artemis AC Corp.

X4 Pharmaceuticals, Inc.

and

Österreichische Forschungsförderungsgesellschaft mbH

1. PARTIES TO THE AGREEMENT

This Settlement Agreement ("***Agreement***") is concluded between the following Parties ("***Parties***"):

(a) ARSANIS Biosciences GmbH, a company incorporated under the laws of Austria, having its statutory seat in Vienna and its registered office at Helmut-Qualtinger-Gasse 2, 1030 Vienna, registered with the Austrian commercial register under number FN 354305 m ("**Arsanis AT**");

(b) Arsanis, Inc. a company formed under the laws of the State of Delaware, having a principal place of business and its registered office at 950 Winter Street, Suite 4500, Waltham, MA02451, United States ("**Arsanis Inc.**");

(c) Artemis AC Corp a company formed under the laws of the State of Delaware, having a principal place of business and its registered office at 950 Winter Street, Suite 4500, Waltham MA 02451, United States ("**Artemis**");

(d) X4 Pharmaceuticals, Inc. a company formed under the laws of the State of Delaware, having a principal place of business and its registered office at 955 Massachusetts Ave, Cambridge, MA 02139, United States ("**X4**"); and

(e) Österreichische Forschungsförderungsgesellschaft mbH a company incorporated under the laws of Austria, having its statutory seat in Vienna and its registered office at Sensengasse 1, 1090 Vienna, registered with the Austrian commercial register under number FN 252263 a ("**FFG**");

2. INTRODUCTION

2.1 Arsanis Inc. is a clinical-stage biopharmaceutical company focused on applying monoclonal antibody immunotherapies to address serious infectious diseases.

2.2 Arsanis AT is a wholly owned subsidiary of Arsanis Inc. focusing on discovery research.

2.3 Artemis, a wholly owned subsidiary of Arsanis Inc., and Arsanis Inc. have entered into a merger agreement with X4, pursuant to which Artemis will merge with and into X4, as a result of which X4 will become a wholly owned subsidiary of Arsanis Inc. (the "**Merger**"). The Merger is expected to close on or about 12 March 2019, subject to the approval of the stockholders of the companies involved, such shareholder approval is expected to be obtained on or before 11 March 2019.

2.4 FFG is the national funding agency for industrial research and development in Austria, its shares are held by the Republic of Austria.

2.5 Arsanis AT has entered into the following subsidy agreements (the "**Subsidy Agreements**") with FFG and has received loans and grants from FFG under these agreements:

Contract No.	Contract Date	Funding Period	Subsidies provided under the Contract
832915	1 July 2011	13 May 2011 – 30 April 2012	**loan and grant**
837128	2 July 2012	1 May 2012 – 30 April 2013	**loan and grant**
841918	1 July 2013	1 May 2013 – 31 January 2014	**loan and grant**
845382	24 March 2014	1 February 2014 – 31 January 2015	**loan and grant**
838450	28 November 2012	1 August 2012 – 31 July 2015	**grant**
850226	6 July 2015	2 February 2015 – 31 January 2017	**loan**
858392	13 February 2017	1 November 2016 – 31 October 2018	**loan**
840293	25 March 2013	1 February 2013 – 31 March 2014	**loan and grant**
846178	20 May 2014	1 April 2014 – 30 April 2015	**loan and grant**
851485	8 July 2015	1 May 2015 – 30 April 2016	**loan and grant**
856836	27 June 2016	1 May 2016 – 30 June 2017	**loan and grant**

2.6 With letters dated 4 February 2019 to Arsanis AT and Arsanis Inc. FFG alleged that Arsanis AT is in breach of certain conditions of the Subsidy Agreements and claimed, among other things, immediate repayment of the loans and repayment of the grants provided under the Subsidy Agreements as well as the payment of interest applied to each of the loans and grants from the date such amounts were paid by FFG to Arsanis AT.

2.7 Arsanis AT and Arsanis Inc. hold the position that Arsanis AT is not in breach of any conditions of the Subsidy Agreements and consequently do not see any legal basis for FFG's claims.

3. SETTLEMENT

Subject to the terms and conditions of this Agreement, Arsanis AT and FFG wish to enter into a full and final settlement with regard to the subject matter described under Section 2.5 to 2.7 of this Agreement.

The Parties agree as follows:

3.1 Repayment of Loans

Arsanis AT shall repay the loans granted under the Subsidy Agreements until 30 June 2021 in accordance with the following amended (accelerated) payment schedule:

Contract No.	Principal Loan Amount in EURO	Original Due Date	New Due Date	New Repayment Schedule		
				31 March 2019	30 June 2020	30 June 2021
832915	515.357	30 June 2020	**30 June 2020**		515.357	
837128	580.000	30 June 2020	**30 June 2020**		580.000	
841918	1.244.928	30 June 2020	**30 June 2020**		1.244.928	
845382	1.634.932	30 June 2020	**30 June 2020**		1.634.932	
850226	1.500.000	31 March 2022	**31 March 2019**	1.500.000		
858392	750.000	31 March 2023	**31 March 2019**	750.000		
840293	530.693	30 June 2022	**30 June 2021**			530.693
846178	627.712	30 June 2022	**30 June 2021**			627.712
851485	775.262	30 June 2022	**30 June 2021**			775.262
856836	346.320	31 March 2020	**31 March 2019**	346.320		
				2.596.320	3.975.217	1.933.667

Total Amount to be repaid EUR 8.505.204

In case of a default of Arsanis AT with payments under the accelerated payment schedule FFG shall provide Arsanis AT with a reminder giving Arsanis AT a minimum 14 (fourteen) day grace period to effect the outstanding payment. A copy of such letter shall be sent to Arsanis Inc.

3.2 Interest

The regular interest to be paid in connection with the loans shall be paid by Arsanis AT in accordance with the provisions of and at the interest rates agreed in the Subsidy Agreements. Such interest shall accrue for each loan until its respective date of actual repayment. The interest shall be paid by Arsanis AT upon receipt of an invoice from FFG on a half-yearly basis and any remaining amount of interest at the date of actual repayment of the respective loan. Such invoice shall provide for a minimum payment term of two (2) weeks. FFG has the right to collect interest by auto-debiting a bank account of Arsanis AT for all interest amounts, consistent with its current practice. FFG will inform Arsanis AT at the time of sending the respective invoice in case it does not intend to exercise the latter right.

In case of a default of Arsanis AT with interest payments FFG shall provide Arsanis AT with a reminder giving Arsanis AT a minimum 14 (fourteen) day grace period to effect the outstanding interest payments. A copy of such letter shall be sent to Arsanis Inc.

3.3 Cash Balance

Beginning from a date that is no more than fourteen (14) days from the closing of a financing of Arsanis Inc. following the closing of the Merger between X4 and Artemis, but in any event from 30 April 2019, Arsanis AT shall maintain a cash balance equal to at least 70 (seventy) per cent of the then current total principal amount of FFG loans outstanding at any point of time until all principal amounts of loans are fully repaid. The cash balance shall be maintained by Arsanis AT in a bank account held with an Austrian bank.

3.4 Austrian Site

Arsanis AT shall maintain an Austrian site with a minimum number of full-time equivalents of eight (8) employees until at least 31 December 2021. In case of employees leaving Arsanis AT, either because of an employee's resignation or because of dismissal, Arsanis AT is granted a reasonable time period for replacement of such employees. FFG acknowledges that such reasonable time period may vary from time to time depending on the position and qualification of the respective employee(s), but that such time period shall not exceed three (3) months save in case of a specialist skilled employee's resignation or dismissal for cause in which case Arsanis AT shall, promptly following an unsuccessful lapse of such three (3) months period, submit a report to FFG setting out which efforts Arsanis AT has undertaken to seek a suitable replacement employee(s).

3.5 Intellectual Property

The intellectual property (including but not limited to patents, utility patents, designs, trademarks, trade names, domain names, logos, copyright, rights under license agreements, recipes, formulas and know-how) ("**IP**") which generated or may be generated (if any) from or in relation to the projects funded by FFG under the Subsidy Agreements (which, for the sake of clarity, the parties agree does not include ASN500) shall remain part of the assets of Arsanis AT at least until 31 December 2021. While Arsanis AT is not under any obligation to continue to develop such IP, it agrees that in the event that it licenses or provides rights to such IP to any third party it will receive arm's length compensation as consideration for at least the period up to 31 December 2021.

FFG is aware that Arsanis Inc. has entered in an option and license agreement with subsidiaries of Bravos Biosciences, LLC for the ASN200 and ASN300 programs and confirms that this licensing (as disclosed to FFG) does not constitute a violation of the above-mentioned obligation or any other obligation between Arsanis AT, Arsanis Inc. and FFG, including under the Subsidy Agreements.

FFG was informed by Arsanis AT by way of final report regarding the ASN400 project (submitted to FFG by Arsanis AT on 1 March 2018) that Arsanis AT has abandoned the respective project and confirms the abandonment does not constitute a violation of the above-mentioned obligation or any other obligation, including under the Subsidy Agreements.

Further, FFG was informed by Arsanis AT that Arsanis AT has discontinued its Phase 2 clinical trial of ASN100 for the prevention of *S. aureus* pneumonia in high-risk, mechanically ventilated patients and confirms that such action does not constitute a violation of the above-mentioned obligation or any other obligation, including under the Subsidy Agreements**.**

3.6 Reporting

Arsanis AT shall provide FFG with a bank account excerpt, balance sheet and statement of operations (P&L statement) of Arsanis AT (each dated as of the end of the respective calendar quarter) on a quarterly basis. The bank account excerpt shall be sent within five (5) days after the end of the respective calendar quarter, and the other above reports shall be sent to FFG within five (5) days after Arsanis Inc. files its required quarterly and annual reports on Forms 10-Q and 10-K, respectively, with the United States Securities and Exchange Commission.

In case of a failure of Arsanis AT to deliver any of the bank account excerpts or any of the reports according to the foregoing paragraph, FFG shall provide Arsanis AT with a reminder giving Arsanis AT a minimum fourteen (14) day grace period to provide such excerpts or reports. A copy of such letter shall be sent to Arsanis Inc.

3.7 Commitments of Arsanis Inc.

Arsanis Inc. shall procure the timely transfer of sufficient funds to Arsanis AT in order to enable Arsanis AT to ensure the minimum cash balance requirement in accordance with Section 3.3 of this Agreement can be met by Arsanis AT at any given time.

Arsanis Inc shall use its best efforts to enable Arsanis AT to comply with the obligations provided for under Section 3.1 to 3.6 of this Agreement and shall refrain from any instructions and measures that might endanger such compliance.

3.8 Commitments of Artemis and X4

Artemis and X4 confirm that they are aware of the obligations and commitments of Arsanis AT and Arsanis Inc. provided for under Section 3.1 to 3.7 of this Agreement. Each of them shall use commercially reasonable efforts to enable Arsanis AT and Arsanis Inc. to comply with their obligations provided for under Section 3.1 to 3.7 of this Agreement, which will not include actions that cause a breach or event of default under X4's existing loan facilities, and shall refrain from any instructions and measures that might endanger such compliance.

3.9 Event of Default

In the event of a breach (respectively a continuing breach despite the granting and the lapse of a grace period if and where a grace period is expressly foreseen under this Agreement) by Arsanis AT and/or Arsanis Inc. of any of the obligations, commitments and undertakings stipulated in Section 3.1 to 3.5, with respect to Section 3.6 only as far as it is related to the bank account excerpts and with regard to the first paragraph of Section 3.7 of this Agreement, FFG shall be entitled to declare any of the outstanding loan repayments as stipulated under Section 3.1 or parts thereof due for immediate repayment.

Any further rights FFG might have under any of the Subsidy Agreements remain unaffected.

3.10 Waiver of claims

Except for the claims for repayment of the loans and regular interest according to the payment schedule under Section 3.1 and Section 3.2 of this Agreement, FFG waives all claims against Arsanis AT and Arsanis Inc., including those set forth in the letters described in Section 2.6, in particular any claims for repayment of grants and interest exceeding such regular interest, under the suspensive condition and provided that Arsanis AT and Arsanis Inc. comply with the obligations and commitments according to Sections 3.1 to 3.7 of this Agreement. FFG will not pursue such claims as long as an Event of Default according to Section 3.9 of this Agreement has not occurred.

Subject to the fulfillment of Arsanis AT's obligations and commitments according to Sections 3.1 to 3.5 of this Agreement, FFG releases with effect as of 31 December 2021 (i) Arsanis AT from all obligations and claims arising from and in relation to the Subsidy Agreements, (ii) Arsanis Inc. from all obligations vis-a-vis FGG and claims of FFG arising from and in relation to Arsanis, Inc.'s commitments provided under this Agreement and provided for under any other documents in favor of Arsanis AT in effect as of the date of this Agreement and (iii) Artemis and X4 from all obligations and claims arising from and in relation to their commitments provided under this Agreement.

3.11 No further payouts under the Subsidy Agreements:

Arsanis AT shall not be entitled to any further payouts of loans and grants under the existing Subsidy Agreements.

4. GOVERNING LAW AND JURISDICTION

4.1 This Agreement shall be governed by and construed in accordance with the laws of Austria excluding Austrian conflict of law rules.

4.2 All disputes or claims arising out of or in connection with this Agreement, including disputes relating to its validity, breach, termination or nullity, shall be finally settled under the Rules of Arbitration (Vienna Rules) of the Vienna International Arbitral Centre (VIAC) of the Austrian Federal Economic Chamber by one or three arbitrators appointed in accordance with the said Rules. The place of arbitration shall be Zurich, Switzerland. The language of the arbitration shall be English.

5. STAMP DUTY

5.1 The Parties hold the view that this Agreement falls under the exemption from stamp duties according to Sec. 14 (2) of the Act on the Establishment of the Austrian Research Promotion Agency (Forschungsförderungs-gesellschaftsgesetz).

5.2 In case Austrian tax authorities take a different view the stamp duties arising from this Agreement shall be equally borne by FFG and Arsanis AT in equal parts.

6. EFFECTIVENESS AND EXECUTION/MISCELLANEOUS:

6.1 This Agreement shall become effective upon signature by all Parties.

6.2 It shall be executed in five (5) originals of which each Party shall retain one. Each Party has the requisite power and authority to execute and perform its respective obligations under, this Agreement. Each of the undersigned declares in lieu of oath to have the requisite power and authority to enter into this Agreement with binding effect upon the respective Party.

6.3 The Parties agree to keep this Agreement, the content of this Agreement and any information provided for under Section 3.6 of the Agreement confidential and not to disclose it to third parties. The aforementioned obligation does not apply (i) with regard to a disclosure to its group companies to the extent reasonably necessary to perform its obligations under this Agreement, (ii) with regard to information that is already in a public domain for reasons other than the breach of this Agreement and (iii) in case and to the extent a Party (x) reasonably requires disclosure in order to preserve or enforce its rights and

claims under or in relation to this Agreement or (**y**) is obliged to disclose the existence or the content of this Agreement under applicable laws, a binding order of a competent court, a competent (regulatory) authority or tribunal or to the Austrian government or its agencies, including but not limited to the Austrian Government Accountability Office (*Rechnungshof*). For clarity, Arsanis Inc. is obligated to disclose the entry into this Agreement and to file it in a public filing with the United States Securities and Exchange Commission promptly upon execution of the Agreement.

6.4 This Agreement shall be binding on each Party's successors and assigns.

6.5 This Agreement may only be amended or terminated, and any provision hereof may only be waived, in writing signed by all of the parties hereto.

6.6 This Agreement, along with the Subsidy Agreements as altered hereby, supersedes any and all prior or contemporaneous oral and/or written agreements between Arsanis AT, Arsanis Inc., Artemis and X4, on the one hand, and FFG, on the other hand, and sets forth the entire agreement between Arsanis AT, Arsanis Inc., Artemis and X4, on the one hand, and FFG, on the other hand.

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SIGNATURE PAGE

ARSANIS Biosciences GmbH

/s/ Michael P. Gray
Name: Michael P. Gray
Position: Managing Director
Date: 8 March 2019

Arsanis, Inc.

/s/ Michael P. Gray
Name: Michael P. Gray
Position: Chief Executive and Chief Financial Officer
Date: 8 March 2019

Artemis AC Corp.

/s/ Michael P. Gray
Name: Michael P. Gray
Position: Director
Date: 8 March 2019

X4 Pharmaceuticals, Inc

/s/ Paula Ragan, Ph.D.
Name: Paula Ragan, Ph.D.
Position: President and Chief Executive Officer
Date: 3/8/2019

Österreichische Forschungsförderungsgesellschaft mbH

/s/ Dr. Egerth /s/ Dr. Pseiner
Name: Dr. Egerth
Dr. Pseiner
Position: Managing Directors
Date: March 8, 2019

Exhibit 99.1

DESCRIPTION OF THE BUSINESS OF X4 PHARMACEUTICALS, INC.

Overview

We are a clinical-stage biopharmaceutical company focused on the discovery, 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. 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 (X4P-001), has completed a Phase 2 clinical trial in patients with Warts, Hypogammaglobulinemia, Infections, and Myelokathexis, or WHIM, syndrome, which is a PI. We plan to initiate a Phase 3 pivotal clinical trial of mavorixafor for the treatment of patients with WHIM syndrome in the second quarter of 2019 and report top-line data from this trial in 2021. Beyond WHIM syndrome, we plan to initiate a Phase 1 clinical trial of mavorixafor in another PI, severe congenital neutropenia, or SCN, and a Phase 1/2 clinical trial of mavorixafor in Waldenström macroglobulinemia, or WM, in 2019. We expect to report data from the SCN trial in the middle of 2020 and data from the WM trial in the second half of 2020.

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. Specifically, WHIM syndrome, one of these PIs, is caused by a mutation in the CXCR4 receptor that results in the receptor's signaling to remain "on" longer than normal. This excessive signaling immobilizes white blood cells, including neutrophils and lymphocytes, in the bone marrow where they are produced and dramatically reduces their ability to move into the blood and perform effective immunosurveillance. WHIM patients often have chronic neutropenia and lymphopenia (abnormally low neutrophils or lymphocytes, respectively) along with increased susceptibility to infections and certain cancers. We sponsored a preliminary independent market research study conducted by a third-party research firm that surveyed 212 physicians in the United States, who reported that over 1,700 patients have either genetically confirmed or are highly suspected to have WHIM syndrome in the United States alone. Based on this study, we estimate there are more than 1,000 genetically confirmed WHIM patients in the United States. Currently, there are no approved therapies for the treatment of WHIM syndrome and care is limited to the symptomatic treatment of the different manifestations of this disease.

Mavorixafor, our lead product candidate, 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. Mavorixafor has completed an open-label, dose escalation Phase 2 clinical trial in patients with WHIM syndrome. In the Phase 2 trial, we observed that mavorixafor increased neutrophil and lymphocyte counts and was associated with improvement in certain signs and symptoms of WHIM syndrome. The increase in absolute neutrophil counts, or ANCs, was observed in the seven evaluable patients in the trial, with five of seven patients (71%) exceeding the pre-defined target threshold of 600/μL for ANCs. Similarly, we observed that mavorixafor increased absolute lymphocyte counts, or ALCs, with six of seven patients (86%) exceeding the pre-defined target threshold of 1,000/μL for ALCs. These thresholds of 600/μL for ANCs and 1,000/μL for ALCs correspond to the National Cancer Institute's adverse event grading system, which lists ANCs below 500/μL to be severe or life threatening and ALCs of 1,000/μL within the range of healthy individuals. In the Phase 2 trial, mavorixafor was not associated with any treatment-related serious adverse events and was observed to be well tolerated in daily doses of up to 400 mg for durations of up to 400 days. Additionally, patients experienced improved infection rates, as reported by patients and the trial investigators. Significant and visible reductions in wart lesions were also reported in a patient with a history of untreatable severe wart lesions. To date, over 150 patients in clinical trials have been dosed with mavorixafor which has demonstrated a favorable tolerability profile. Based on the clinical data generated to date and our discussions with the U.S. Food and Drug Administration, or FDA, we have finalized the clinical trial protocol for our Phase 3 pivotal clinical trial of mavorixafor for the treatment of patients with WHIM syndrome and expect to commence the clinical trial in the second quarter of 2019 and report top-line data in 2021.

We believe that mavorixafor's approach through antagonism of the CXCR4 receptor has been validated by the FDA-approved product plerixafor for injection (marketed as Mozobil). Plerixafor is a CXCR4 antagonist that has been shown to induce white blood cell mobilization and is used for short-term treatment in preparation for stem-cell transplants. In a published investigator-sponsored pilot study of WHIM patients, twice-daily injections of plerixafor demonstrated increased white blood cell counts, including ANCs and ALCs, and reduced infections and wart lesions. We believe that this data validates CXCR4 antagonism as a mechanism of action for treating WHIM syndrome. However, plerixafor is not approved for the treatment of WHIM syndrome and we are not aware of any plans to develop it as a treatment for WHIM syndrome. In addition, plerixafor is only available in injectable form and its use is limited to four days of treatment. We believe that mavorixafor, which is being developed as an oral, once-daily treatment, has the potential to provide less invasive dosing and better patient compliance for life-long use in WHIM patients.

In addition to our initial focus on WHIM syndrome, we believe that the biological rationale and available data on mavorixafor support potential therapeutic benefits across a broad range of PIs, including SCN, and certain lymphomas, such as WM. SCN is a rare blood disorder that is characterized by abnormally low levels of certain white blood cells and has an estimated prevalence of approximately 2,000 to 3,000 persons in the United States and European Union. WM is a rare form of non-Hodgkin's lymphoma, which has an estimated prevalence of over 13,000 persons in the United States and European Union, at annual incidence rates of 1,000 to 1,500 in the United States and approximately 1,800 in the European Union. We plan to initiate a Phase 1 clinical trial of mavorixafor in SCN and a Phase 1/2 clinical trial of mavorixafor in WM in 2019. We expect to report data from the SCN trial in the middle of 2020 and data from the WM trial in the second half of 2020. We are also currently assessing mavorixafor in the Phase 2a portion of an open-label Phase 1/2 clinical trial for the treatment of patients with clear cell renal cell carcinoma, or ccRCC, in combination with axitinib, an FDA approved small molecule tyrosine kinase inhibitor. Final data from this trial is expected in the second half of 2019. We intend to pursue a strategic collaboration for future development and potential commercialization of mavorixafor in ccRCC and potentially other immuno-oncology indications.

We are also developing X4P-002, a CXCR4 antagonist that has unique properties that we believe will enable it to penetrate the blood-brain barrier and provide appropriate therapeutic exposures to treat brain cancers, including glioblastoma multiforme, or GBM. We are also developing X4P-003, a second generation molecule designed to have an enhanced pharmacokinetic profile relative to mavorixafor, potentially enabling improved patient compliance and ease of use to better serve patients suffering from chronic rare diseases. Both of these programs are in preclinical development.

Our leadership team has considerable experience with research, development and commercialization of therapies to treat rare diseases, including therapies that target chemokine pathways. Paula Ragan, Ph.D., our founding Chief Executive Officer, previously held leadership roles at Genzyme, a Sanofi company. Dr. Ragan led the licensing of the CXCR4 antagonist portfolio from Genzyme and coordinated all phases of the transfer of the knowledge and know-how needed to launch our company. Our co-founder, Renato Skerlj, Ph.D., is an inventor of plerixafor, the only FDA-approved CXCR4 antagonist (for injection only) as well as ertapenem, an anti-bacterial approved by the FDA in 2001. Two members of our Board of Directors also have deep roots in our differentiated chemokine approach, including Gary J. Bridger, Ph.D., who was responsible for the discovery and development of plerixafor as a co-founder and Chief Scientific Officer of AnorMED Inc., until the company's acquisition by Genzyme in 2006, and Michael S. Wyzga, Chairman of our Board of Directors, who was the Chief Financial Officer of Genzyme during the approval, global launch and subsequent commercialization of plerixafor. We believe the experience of our leadership team provides our company with unique insights into product development and commercialization processes and the identification of other opportunities involving CXCR4 biology.

In October 2018, we received Orphan Drug Designation from the FDA for mavorixafor for the treatment of WHIM syndrome. If mavorixafor is approved for WHIM syndrome, this would provide mavorixafor with up to seven years of market exclusivity for this indication. As of March 15, 2019, we owned or exclusively licensed 12 issued U.S. patents, 10 pending U.S. non-provisional patent applications, five pending U.S. provisional patent applications and approximately 120 PCT and foreign patents and patent applications. We have exclusively licensed a portfolio of patents and patent applications that includes claims to mavorixafor-related molecules, including a granted U.S. patent with composition of matter claims to the new chemical entity defining mavorixafor. This patent is expected to expire in December 2022, excluding possible patent extensions of up to five years. Additionally, we have filed several patent applications for our wholly owned intellectual property portfolio, which includes additional composition of matter claims for our mavorixafor product formulation. If granted, these patent filings are expected to expire in 2036 and beyond.

Prior to March 13, 2019, we were a clinical-stage biopharmaceutical company known as Arsanis, Inc., or Arsanis, that had historically been focused on applying monoclonal antibody immunotherapies to address serious infectious diseases. Arsanis was originally incorporated in the State of Delaware in August 2010. On March 13, 2019, we completed our business combination with X4 Therapeutics, Inc., formerly X4 Pharmaceuticals, Inc., or X4, in accordance with the terms of an Agreement and Plan of Merger, dated as of November 26, 2018, as amended on December 20, 2018 and March 8, 2019, or the Merger Agreement, that we entered into with X4 and Artemis AC Corp., a Delaware corporation and our wholly owned subsidiary, or Merger Sub. Pursuant to the terms of the Merger Agreement, Merger Sub merged with and into X4, with X4 continuing as our wholly owned subsidiary and the surviving corporation of the merger, which we refer to as the Merger. At the closing of the Merger, we issued shares of our common stock to X4 stockholders based on an agreed upon exchange ratio, and each option or warrant to purchase X4 capital stock became an option or warrant, respectively, to purchase our common stock, subject to adjustment in accordance with the agreed upon exchange ratio. Following the closing of the Merger, we effected a 1-for-6 reverse stock split of our common stock, our name was changed to X4 Pharmaceuticals, Inc., the business of X4 became our business, and we became a clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of novel therapeutics for the treatment of rare diseases. In connection with the closing of the Merger, our stock began trading on the Nasdaq Capital Market under the symbol "XFOR" on March 14, 2019. In addition to our corporate headquarters located in Cambridge, Massachusetts, we also have a research and development team located in Vienna, Austria.

Our Strategy

Our goal is to discover, develop and commercialize novel therapeutics, based on established CXCR4 biology, for the treatment of rare diseases, including a broad range of PIs and cancer. The key tenets of our business strategy to achieve this goal include:

- ***Advance our lead rare disease program through pivotal clinical development in WHIM syndrome.*** We have completed a Phase 2 clinical trial of mavorixafor in patients with WHIM syndrome. In the completed Phase 2 trial, we achieved clinical proof-of-concept for mavorixafor in WHIM syndrome, observing a clinically meaningful increase in neutrophil and lymphocyte counts and a favorable tolerability profile. Based on clinical data to date and our discussions with the FDA, we plan to initiate the Phase 3 pivotal clinical trial in the second quarter of 2019 and expect to report top-line data from the trial in 2021.
- ***Drive community awareness of and support for WHIM syndrome and build patient registries.*** We are highly focused on our efforts to help build awareness of underserved serious rare diseases, such as WHIM syndrome, among patients, physicians and their support systems. Based on the preliminary independent market research study that we sponsored, we believe that there are more than 1,000 genetically confirmed WHIM patients in the United States. In addition to our sponsored market research and outreach efforts, we have partnered with key patient foundations and registries, including the Jeffrey Modell Foundation, University of Washington, Immune Deficiency Foundation and Hopitaux Universitaires Est Parisien (Trousseau La Roche-Guyon), with the objective of increasing awareness of WHIM syndrome and improving patient diagnosis. In April 2018, we initiated a 300 patient prospective screening study, in collaboration with the Jeffrey Modell Foundation, to establish a systematic diagnostic approach for WHIM syndrome and to support the identification of WHIM patients by combining clinical features and genetic testing. To supplement these efforts, we have also deployed a field force of Medical Science Liaisons, or MSLs, in the United States to further drive education and awareness of WHIM syndrome. We plan to leverage our relationship with our partner organizations and patient registries to provide us with access to patients for clinical trial enrollment, which we believe will provide us with a significant advantage in rare disease drug development where patients are often hard to locate and recruit.

- ***Advance additional indications for mavorixafor.*** Our goal is to maximize the commercial potential of mavorixafor. Given aberrant functioning of the CXCR4 receptor is implicated in a variety of PIs, we believe mavorixafor has the potential to offer therapeutic benefits to patients suffering from certain of the 250 already defined PIs beyond WHIM syndrome. The next PI on which we believe mavorixafor can have a meaningful therapeutic effect is SCN and we intend to initiate a Phase 1 trial of mavorixafor in SCN in 2019. We believe mavorixafor may also have the potential to treat certain blood cancers, including WM, where regulation of the CXCR4 receptor has been shown to play a key role in treatment resistance and cancer progression. We intend to initiate a Phase 1/2 trial of mavorixafor in WM in 2019.
- ***Advance earlier-stage product candidates and leverage insights into CXCR4 biology to further expand our pipeline.*** Our second product candidate, X4P-002, is currently in preclinical development and is designed to selectively antagonize CXCR4. In preclinical studies, we have observed that X4P-002 has the ability to penetrate the blood-brain barrier and we believe X4P-002 has the potential to provide appropriate therapeutic exposures for GBM. Our third product candidate, X4P-003, is currently in preclinical development and is a second generation peripherally acting CXCR4 antagonist, with an enhanced pharmacokinetic profile relative to mavorixafor. We believe that these improved properties could allow X4P-003 to enable improved patient compliance and ease of use to better serve patients suffering from chronic rare diseases. We intend to leverage our insights into CXCR4 biology and our research capabilities to discover and develop additional product candidates with potential to offer meaningful clinical benefit.
- ***Independently commercialize our product candidates in certain indications and geographies where we believe we can maximize value.*** Given the potential of our product candidates to treat a wide variety of diseases, we believe that it will be important to maintain discipline with respect to our development and commercialization efforts. We plan to independently develop product candidates in indications, including rare diseases, where we believe there is a well-defined clinical and regulatory approval pathway and that we believe we can commercialize those product candidates successfully, if approved. In addition, we may seek to enter into strategic collaborations around product candidates, disease areas or geographies that we believe could benefit from the resources of either larger biopharmaceutical companies or those specialized in a particular area of relevance.
- ***Seek strategic collaborations for mavorixafor in immuno-oncology indications.*** We intend to pursue strategic collaborations for future development and potential commercialization of mavorixafor in immuno-oncology indications in order to maximize the value of that asset while we maintain our focus on developing mavorixafor for rare diseases. Given what we believe is the significant potential of mavorixafor in immuno-oncology, we believe future development and potential commercialization is better served by the resources of larger biopharmaceutical companies. We are currently assessing mavorixafor in the Phase 2a portion of an open-label Phase 1/2 clinical trial for the treatment of patients with ccRCC in combination with axitinib. Previously, we reported interim data from our Phase 1b melanoma clinical trial in which we observed single-agent activity in the tumor microenvironment, or TME, with meaningful increases in CD8+ T-cells.

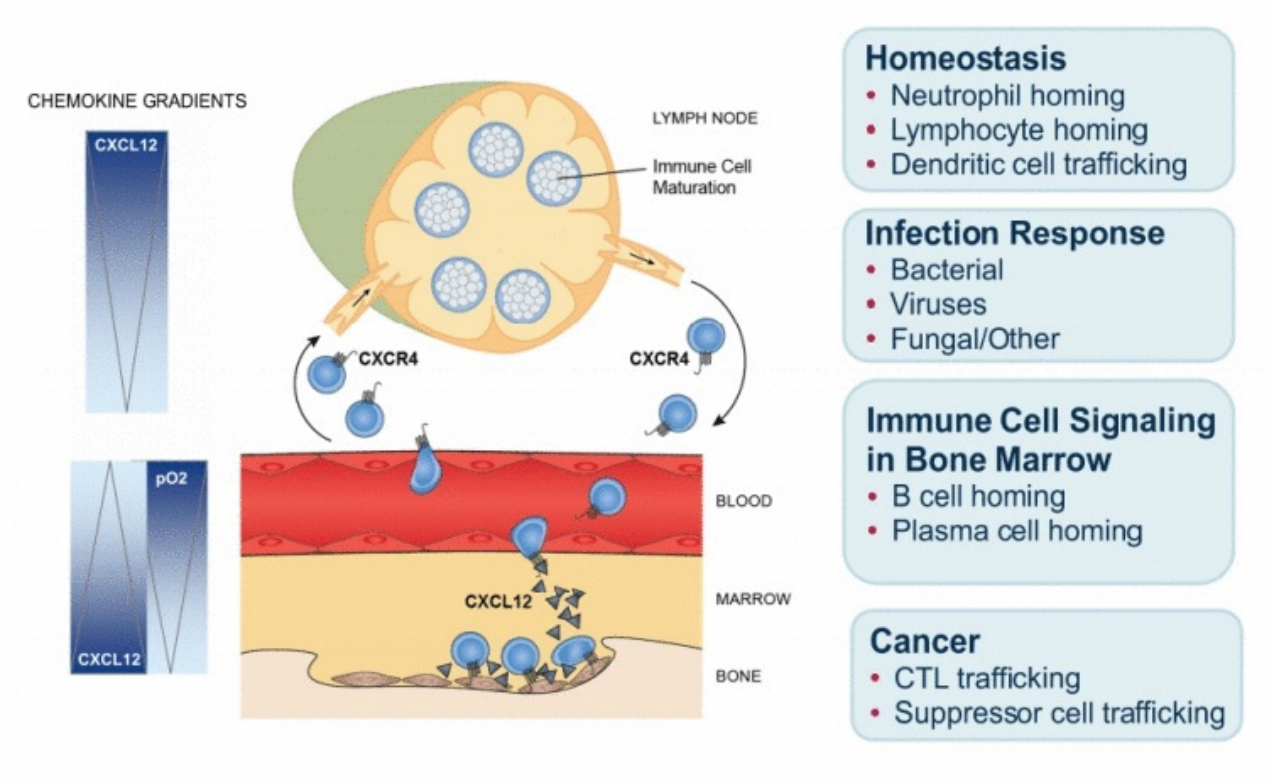
Our Approach

We are focused on restoring healthy immune system function by developing selective, oral, small molecule antagonists of chemokine receptor CXCR4 to treat rare diseases, including PIs and cancer. Chemokines are signaling proteins that guide the migration of immune cells within the body by binding to receptors on the surface of target cells. When the chemokine receptor CXCR4 is stimulated by its only chemokine ligand, CXCL12, it plays a key role in enabling the trafficking of immune cells and effective immunosurveillance. When the CXCL12/CXCR4 pathway is overstimulated, immune cells become immobilized, which can lead to immunosuppression.

In the case of PIs, such as WHIM syndrome, overstimulation of the pathway is caused by mutations in the CXCR4 receptor, which results in premature truncations in the CXCR4 protein and causes excessive signaling of the receptor despite normal levels of the ligand CXCL12. This excessive “on” signaling caused by the “gain of function” mutations immobilizes white blood cells in the bone marrow where they are produced, and dramatically decreases their ability to move into the blood and perform immunosurveillance. In other diseases, such as certain types of cancer, the CXCL12/CXCR4 pathway has been found to broadly play a role in disrupted immune cell trafficking in the TME, where there often exists an abnormally high concentration of the ligand CXCL12. Evidence also suggests that the pro-tumor signals between tumor cells and cancer associated fibroblasts occur partly through chemokine signaling, including through the over-production of CXCL12.

We are developing oral allosteric antagonists of CXCR4 in order to block overstimulation of the CXCL12/CXCR4 pathway. Allosteric antagonists bind to a portion of the receptor away from the ligand binding pocket. Allosteric binding results in a conformational change in the receptor that decreases the ligand's ability to bind and reduces ligand-dependent signaling. We believe allosteric inhibition can robustly block the signaling of the CXCR4 receptor, either when the receptor is mutated, as in the case in PIs and WM, or in the presence of high concentrations of CXCL12, as in the case of many solid tumors. Ultimately, the inhibition of the CXCL12/CXCR4 signaling has the potential to improve immune cell trafficking and immunosurveillance. This is depicted in Figure 1.

Figure 1: CXCL12/CXCR4 and Immune System Responses.

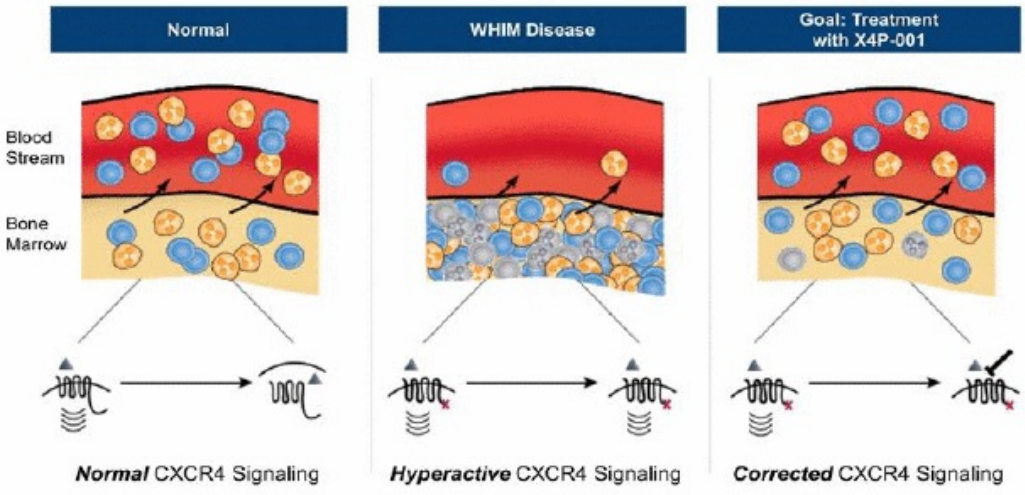


Rationale in Primary Immunodeficiencies

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, WHIM syndrome is one of a number of PIs that are caused by the improper trafficking of immune cells. WHIM syndrome is a rare genetic disease that results from a "gain of function" mutation in the single gene that encodes for the CXCR4 receptor, with the first such mutation identified in 2003. Since then, a total of nine different CXCR4 mutations have been identified as causing WHIM syndrome. These mutations cause premature truncations in the protein, causing the receptor to remain "on" longer than normal, which results in the retention of white blood cells in the bone marrow where they are produced, and leads to the chronic peripheral neutropenia and lymphopenia that is the observed clinical hallmark of WHIM syndrome.

Figure 2 illustrates the mutation in the CXCR4 receptor leading to abnormal signaling and retention of white blood cells in the bone marrow that occurs in WHIM patients. Figure 2 also depicts our approach to blocking this abnormal signaling with a CXCR4 antagonist, enabling the white blood cells to release into the bloodstream, restoring normal immune function. As depicted below, normally the CXCR4 receptor can be internalized into the cell after CXCL12 binds to it, enabling the receptor to be appropriately "recycled" and the signaling to be diminished. In WHIM patients, however, a mutation truncates the intracellular portion of the CXCR4 receptor as shown by the red "x" below, which prevents the post-binding internalization ("normal recycling") of the receptor. As a result, the CXCR4 receptor is maintained on the surface of the cell and is exposed to the ligand, which creates a perpetual "on" signaling and immobilizes the cell. Mavorixafor binds to the mutated CXCR4 receptor in a manner that blocks the receptor from being stimulated by CXCL12 regardless of the presence of the ligand, and results in increased mobilization and trafficking of white blood cells from the bone marrow.

Figure 2. WHIM Syndrome: Genetic Mutations in CXCR4 Create Abnormal Trafficking of White Blood Cells



There are other PIs beyond WHIM syndrome that are also believed to be a result of immune trafficking dysregulation. Like WHIM syndrome, these diseases are often characterized by chronic neutropenia, with neutrophil counts of less than 500 cell/μL, chronic lymphopenia, and increased susceptibility to infections and higher incidence of certain cancers. Similar to WHIM syndrome, SCN is a rare blood disorder characterized by increased risks of infections and cancer due to abnormally low levels of certain white blood cells, including neutrophils and lymphocytes, in the body. Additionally, some sub-types of SCN, such as G6PC3 and GATA2 dysfunction immunodeficiencies, have mechanisms that overlap with mechanisms of the CXCL12/CXCR4 pathway. SCN may be inherited as either an autosomal dominant or an autosomal recessive genetic trait. Additionally, many cases of SCN are the result of spontaneous, random mutations. While CXCR4 mutations have not been established as the

genetic cause of some of these PIs, in clinical trials mavorixafor has been observed to increase neutrophil and lymphocyte counts across all patients (WHIM syndrome and cancer) dosed at or above 300 mg per day. We believe, therefore, that a CXCR4 antagonist may be able to positively impact patient outcomes by directly addressing immune cell trafficking dysregulation and increasing the levels of circulating white blood cells, including neutrophils, to improve immune system function.

Rationale in Lymphomas and Solid Tumor Cancers

WM is a rare form of non-Hodgkin's lymphoma and B-cell lymphoproliferative disorder. The second most frequently mutated gene in WM is CXCR4, which occurs in approximately 30% of WM cases, two-thirds of which are the C1013G variant of this gene, which is the same predominant variant as in WHIM syndrome. In WM, somatic mutations of the CXCR4 receptor in B-cell lineages are associated with activating and pro-survival signaling of tumor cells, as well as the possible acquisition of resistance to current recommended standard of care, ibrutinib, a Bruton tyrosine kinase, or BTK, inhibitor. In WM patients who have been treated with ibrutinib, patients with WHIM syndrome-like mutations have a reduced median progression-free survival, or mPFS, as compared to patients without the somatic WHIM syndrome mutation.

In solid tumors, the TME consists of the tumor cells and cancer associated fibroblasts, or CAFs, each of which overproduce growth factors and chemokines to support immune-suppression and malignant cell proliferation and growth. Evidence suggests that the pro-tumor signals between tumor cells and CAFs occur, in part, through chemokine signaling, including through the over-production of CXCL12. The CXCL12/CXCR4 pathway has been shown to be overstimulated in over 20 solid and blood-derived tumor types. Excessive stimulation of CXCR4 due to high concentrations of CXCL12 influences trafficking immune cells, including myeloid-derived suppressor cells, or MDSCs, CD4+ regulatory T-cells, or Tregs, CD8+ T-cells, and mature dendritic cells. We believe that blocking CXCR4 overstimulation can lead to improved immune cell trafficking and increase the absolute number of CD8+ T-cells, thereby also increasing the ratio of CD8+ T-cells to Tregs in the TME.

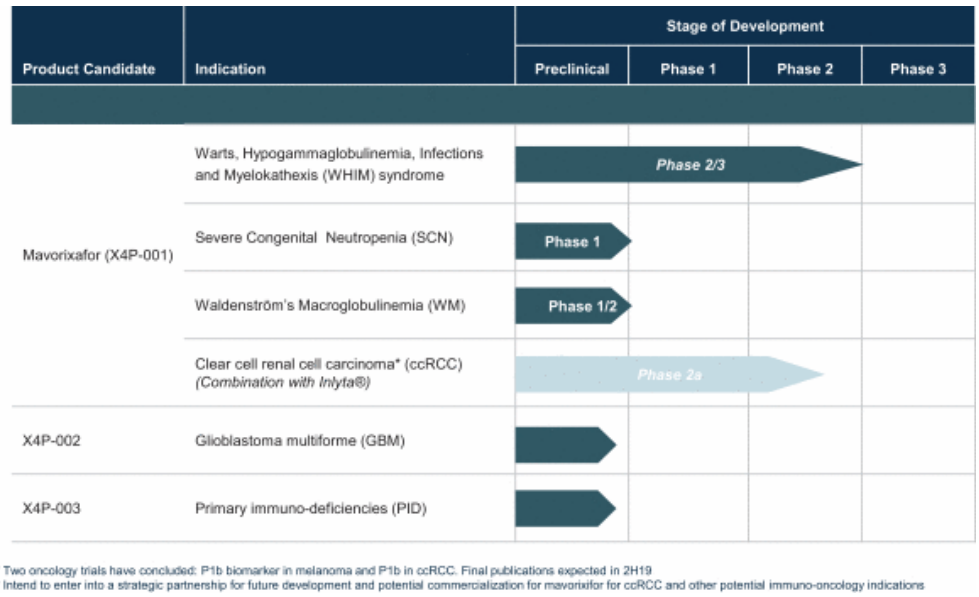
Our Product Candidates

We are developing a pipeline of potentially first-in-class, oral, small molecule CXCR4 antagonists with the potential to address a broad range of rare diseases, including PIs and cancers. Our product candidates are based on a single novel mechanism of allosteric inhibition of the CXCR4 receptor.

Our Rare Disease Programs

Mavorixafor is our lead product candidate and is a potentially first-in-class, oral, allosteric inhibitor of the CXCR4 receptor targeting the correction of abnormal signaling of the receptor and enabling mobilization and trafficking of immune cells into the bloodstream. We have completed a Phase 2 clinical trial in patients with Warts, Hypogammaglobulinemia, Infections, and Myelokathexis, or WHIM, syndrome, a rare congenital disease caused by a mutation in the single gene that encodes for the CXCR4 receptor, and plan to initiate a Phase 3 pivotal clinical trial of mavorixafor for the treatment of WHIM syndrome in the second quarter of 2019. In addition to our initial focus on WHIM syndrome, we believe that the biological rationale and available data on mavorixafor supports potential therapeutic benefits across a broad range of PIs, including severe congenital neutropenia, or SCN. We plan to initiate a Phase 1 clinical trial for mavorixafor in SCN in 2019. We are also advancing mavorixafor in rare blood cancers, with its initial development focused on Waldenström macroglobulinemia, or WM, where dysregulation of the CXCR4 receptor has been shown to play a key role in treatment resistance and cancer progression. We plan to initiate a Phase 1/2 clinical trial for mavorixafor in WM in 2019. We are also currently conducting the Phase 2a portion of an open-label Phase 1/2 clinical trial for mavorixafor in ccRCC and intend to pursue a strategic collaboration for future development and potential commercialization of mavorixafor in ccRCC and other potential immuno-oncology indications.

The following table summarizes key information about our product candidates. We have retained worldwide clinical development and commercialization rights for the product candidates identified below.



Mavorixafor in WHIM Syndrome

We are developing mavorixafor as a potentially first-in-class, oral, allosteric inhibitor of CXCR4 for the treatment of WHIM syndrome. We have achieved clinical proof of concept for mavorixafor in WHIM syndrome where, in the completed Phase 2 clinical trial, we observed clinically meaningful increases in neutrophil and lymphocyte counts and a favorable tolerability profile. Additionally, patients experienced improved infection rates, as reported by patients and the trial investigators. Substantial and visible reductions in wart lesions were also reported in a patient with a history of untreatable severe wart lesions. We plan to initiate the Phase 3 pivotal clinical trial of mavorixafor in WHIM syndrome in the second quarter of 2019 and expect to report top-line data from this trial in 2021.

Background on WHIM Syndrome

The nomenclature for WHIM syndrome is derived from its clinical characteristics: Warts, Hypogammaglobulinemia, Infections, and Myelokathexis. This acronym, however, does not reflect the broad spectrum of disease manifestations that WHIM patients experience. WHIM syndrome is a rare genetic PI that results from a "gain of function" mutation in the single gene that encodes for the CXCR4 receptor, with the first such mutation identified in 2003. Since then, a total of nine different CXCR4 mutations have been identified as causing WHIM syndrome. These mutations cause premature truncations in the CXCR4 protein, causing the receptor to remain in an "on" state longer than normal, resulting in compromised immune cell trafficking and surveillance.

WHIM patients are typically characterized by having chronic, critically low white blood cell counts, including neutrophils and lymphocytes, which are necessary to mount a healthy immune response to bacterial and viral infections. WHIM patients also sometimes present with warts related to infection with the Human Papilloma Virus, or HPV, and/or low immunoglobulin, or IG, levels, also known as hypogammaglobulinemia. IGs are key proteins that help enable immune responses. In addition, bone marrow aspirates of patients with WHIM syndrome show a "hyper-dense" population of pre-apoptotic immune cells in the bone marrow, which is known as myelokathexis. These conditions reduce the body's ability to achieve a healthy immune response. For a diagnosis of WHIM syndrome, all four classic characteristics of warts, hypogammaglobulinemia, infections and myelokathexis, do not need to be present, which complicates the diagnosis of these patients.

Genetic testing is used to definitively diagnose WHIM syndrome by confirming the presence of an autosomal dominant mutation in the CXCR4 receptor where only one mutated gene need be affected to cause this disorder. The diagnosis of WHIM syndrome may occur at any age and about one-half of reported patients were diagnosed as adults, mostly between 18 and 40 years of age, and the other half were diagnosed primarily before or at the age of 12 years. WHIM syndrome does not appear to result from a "founder effect," in which patients are disproportionately segregated in geographic regions due to genetic mutations in a long-ago ancestor, which has thereafter been regionally propagated. WHIM syndrome has been shown typically to have an autosomal dominant pattern of inheritance, which yields a 50% probability of children inheriting the syndrome from an affected parent.

Due to critically low white blood cell counts, patients with WHIM syndrome typically first present with increased susceptibility to repeated bacterial infections, particularly to encapsulated Gram-positive and Gram-negative bacteria, staphylococcus, and mycobacteria. For example, one WHIM patient reported having six episodes of pneumonia before the age of seven years and another WHIM patient reported having one to two episodes of pneumonia every year from infancy until diagnosis at the age of 23 years. Recurrent lung infections, which include bronchitis and pneumonias, in WHIM patients have led to bronchiectasis, a permanent and severe form of lung damage. Additionally, reductions in and loss of hearing due to otitis media, or infections of the ear, have been reported in WHIM patients. Other severe infections such as meningitis have also been reported in WHIM patients and, in rare cases, death due to sepsis. In addition to bacterial infections, WHIM patients exhibit increased frequency and severity of viral infections, especially from common forms of HPV, resulting in warts on the skin and genitalia usually starting in childhood. As a result, WHIM patients have an increased risk of HPV-associated cancers as they age. HPV infections of the genital tract and oropharynx may progress to cervical and head and neck cancers. In some female WHIM patients, epidermal manifestation attributed to HPV may lead to cervical dysplasia and invasive cancer. In addition, HPV-positive oral squamous cell carcinoma has been reported in a patient with WHIM syndrome.

The incidence and prevalence of WHIM syndrome are not well established. We believe this is due to the relatively recent understanding of the underlying genetics of WHIM syndrome, lack of universal or accessible genetic testing, and limited medical education and awareness of the disease, which is in part driven by the lack of available and disease-modifying treatments. The National Organization for Rare Diseases, or NORD, has reported the incidence of WHIM syndrome to be less than one in 1,000,000 based on a single small registry of eight patients in France. Based on a preliminary independent market research study that we sponsored in the United States, which was conducted by a third party research firm, we believe the prevalence of WHIM syndrome worldwide is significantly higher than the incidence statistics implied by the France-based registry. The study solicited input from community-based physicians of different specialties, including physicians focused on non-malignant hematology, immunology, dermatology, pulmonology and infectious diseases, who are known to manage and/or treat patients with WHIM syndrome. The 212 physicians across these specialties in the United States identified to participate in this study reported over 1,700 patients have genetically confirmed or are highly suspected to have WHIM syndrome in the United States alone. The results of this initial study support our estimate of more than 1,000 genetically confirmed WHIM patients in the United States.

WHIM Syndrome Market Awareness and Engagement

We believe that the prevalence of WHIM syndrome worldwide is significantly higher than the reported incidence statistics imply. We are focused on the following priorities to drive awareness of WHIM syndrome and to increase patient and physician engagement:

- ***Build Patient Registries:*** We are building a database registry of WHIM patients by working with physicians and patient organizations such as the Jeffrey Modell Foundation and the Immune Deficiency Foundation, as well as through social media outreach. We plan to use our database to help us engage with physicians who may have patients who could potentially enroll in our planned Phase 3 pivotal clinical trial.
- ***Prospective Screening Study and Registry:*** We are collaborating with the Jeffrey Modell Foundation in a 300-patient prospective screening study to enable genetic confirmation of suspected WHIM patients. The study is designed to support the identification of WHIM patients by combining clinical features and genetic testing. Once patients are identified, we are partnering with their managing physicians to explore the patient's potential participation in our registry database.
- ***U.S. Field Team:*** We have a focused field team of experienced MSLs covering all regions of the United States to help us increase awareness among physicians, and to help identify physicians who are currently treating WHIM patients or who have patients they believe may have WHIM syndrome. Once patients are identified, we partner with them to explore their potential participation in our screening study and registry database. This field team is further driving the education and awareness of WHIM syndrome through targeted physician outreach and by educating these physicians on how to diagnose WHIM syndrome.
- ***Support Patient Ambassadors:*** We have identified patients with WHIM syndrome who have agreed to be ambassadors for this rare disease. We are working with these ambassadors as they partner with informal social networks and patient foundations to form a WHIM syndrome-focused patient organization.

Limited Current Treatment Landscape for WHIM Syndrome

Currently, there are no approved therapies for the treatment of WHIM syndrome. Care is currently limited to the treatment of the different symptoms of WHIM syndrome. The care of WHIM patients is mainly focused on the prevention and management of infections. None of these treatments, however, have been clinically proven to be effective for treating WHIM syndrome nor do they address the underlying cause of this multi-faceted disease, the genetic defect of the CXCR4 receptor. Current symptoms and their limitations are as follows:

- ***Warts:*** The presence of warts in WHIM syndrome is driven by an underlying HPV infection. Standard treatments, such as topical therapies (for example, imiquimod and salicylic acid), cryotherapy and laser therapy, as well as more aggressive approaches, such as cauterization or surgical removal, have been ineffective in providing durable treatment of warts associated with chronic HPV infections. As WHIM patients generally have limited response to vaccines, the HPV vaccine has had limited effectiveness. The number, size and severity of visible warts in WHIM patients can have a significant negative impact on the patient's quality of life and result in social anxiety issues. Left untreated, chronic HPV-infections are also known to increase the risk of cancer. Patients with WHIM syndrome are reported to have more frequent occurrences of difficult to treat HPV-associated cancers, such as head and neck and anogenital cancers.
- ***Hypogammaglobulinemia:*** Intravenous or subcutaneous Ig administration, referred to as IVIg or SCIg, respectively, can be administered to patients with low Ig levels. In WHIM patients, the administration of Ig therapies raises Ig levels, but has shown no impact on circulating leukocytes and limited or no impact on immune responses. Ig treatment of patients with WHIM syndrome is based on empirical and anecdotal evidence, and there are no clinical data demonstrating the efficacy of Ig treatment for WHIM syndrome. Ig treatment also does not treat or protect against HPV-associated symptoms and diseases, such as warts and certain cancers. Furthermore, Ig administration is costly and time consuming.
- ***Infections:*** WHIM patients are given antibiotics to manage infections. Acute infections usually resolve, although we are aware of reports from clinicians citing death due to pneumonia or sepsis in young WHIM patients. Importantly, even with antibiotic use, infections recur more frequently and persist longer in patients with WHIM syndrome. Further, the toll of multiple, chronic infections in WHIM patients has been known to lead to devastating irreversible pathologies such as hearing loss due

to chronic ear infections and bronchiectasis. Patients are sometimes given a granulocyte-colony stimulating factor, or G-CSF, to increase neutrophil counts, but G-CSF has demonstrated little, if any, impact on lymphopenia or the incidence of infections in WHIM patients. In a small registry of eight WHIM patients in France, three of the four patients who received G-CSF continued to have persistent, repeated infections. Side effects of G-CSF include disabling bone pain, which can be more severe in certain age groups. Additional, less common, treatment-limiting complications of chronic G-CSF administration include myelofibrosis and leukemia.

- ***Myelokathexis:*** G-CSF is sometimes used to treat the myelokathexis characteristic of WHIM syndrome to try to increase the number of neutrophils outside of the bone marrow, but G-CSF has no effect on lymphocyte and other types of white blood cells. Side effects of G-CSF can include disabling bone pain, myelofibrosis and leukemia.

While the costs of managing the chronic impact of WHIM syndrome are unknown, the per-patient cost of treating PIs that are similar to WHIM syndrome based on drug costs alone exceeds $100,000 per year in the United States utilizing similar therapies, such as antibiotics, IVIg, SCIg and/or G-CSF, despite the limited effectiveness of these treatments. Beyond these estimated direct costs, other costs associated with direct and indirect management of the disease, such as repeated immunization, physician visits, or hospitalizations, have not been quantified but are likely to be significant. We believe there is a significant need for a treatment targeting the underlying excessive signaling caused by mutations to the CXCR4 receptor, which is the established cause of WHIM syndrome.

Proof-of-concept clinical trials conducted using twice-daily skin injections of a CXCR4 antagonist called plerixafor (marketed as Mozobil) appear to favorably impact the multiple clinical effects of WHIM syndrome. In a Phase 1 clinical trial in three WHIM patients, treatment with twice-daily injections of plerixafor was observed to increase circulating levels of both neutrophils and lymphocytes, decrease incidence of infection, and reduce wart lesions, as well as improve bone marrow morphology. Although we believe plerixafor provides validation for the use of a CXCR4 antagonist for the treatment of this disease, it is not an ideal treatment for WHIM patients given it is an injectable treatment with a short half-life, requiring long-term, twice-daily injections, which are impractical for chronic use. In addition, safety for chronic treatment using plerixafor has not been established to support its approval for long-term use. Plerixafor is not approved for the treatment of WHIM syndrome and we are not aware of any plans to develop it as a treatment for WHIM syndrome.

Our Solution

Mavorixafor Profile

Mavorixafor is a potentially first-in-class, oral, allosteric antagonist of the chemokine receptor CXCR4 designed to correct the immunosuppression resulting from the abnormal receptor signaling caused by mutations in the single gene that encodes the CXCR4, and is, therefore, designed to address the underlying cause of WHIM syndrome. We believe the allosteric inhibition of CXCR4 by mavorixafor will allow for an improved pharmacological profile and favorable side effect profile for the treatment of PIs as compared to other CXCR4 antagonists because mavorixafor can block the activity of mutated CXCR4 receptors rather than directly compete with the ligand. In addition, the observed drug exposure in patients, the 23-hour half-life and the bioavailability of mavorixafor support once-daily oral dosing, which we believe will provide convenient dosing and better patient compliance for life-long use. The manufacturing process for mavorixafor utilizes well-established small molecule chemistry, yielding a potential commercial product that can be supported by specialty pharmacy distribution.

Clinical Development Summary

In October 2018, we received Orphan Drug Designation from the FDA for mavorixafor for the treatment of WHIM syndrome. In 2018, we presented results from our Phase 2 clinical trial in WHIM syndrome and expect to initiate a Phase 3 pivotal clinical trial in the second quarter of 2019 and report topline results from such Phase 3 clinical trial in 2021. In March 2019, we submitted our orphan drug designation request to the European Medicines Agency, or EMA, for mavorixafor for the treatment of WHIM syndrome and we expect to receive such designation in the EMA by mid-2019.

Our Phase 2 Clinical Trial

In January 2017, we initiated a Phase 2 clinical trial of mavorixafor for the treatment of patients with WHIM syndrome. This trial was an open-label, dose-escalation trial in eight WHIM patients conducted at two sites in the United States and Australia pursuant to an IND that we submitted to the FDA in June 2016. The primary objective of the Phase 2 trial was to determine the safety and tolerability of mavorixafor and to determine the dose of mavorixafor for exploration in a Phase 3 pivotal clinical trial. The secondary objective of the Phase 2 trial was to evaluate the potential efficacy of mavorixafor in patients with WHIM syndrome by measuring biomarkers, specifically neutrophil and lymphocyte counts, over 24-hour dosing cycles. The frequency of infections, antibiotic use, hospitalizations, severity of warts lesions, and vaccine titer levels, among other metrics, were also examined. To be included in the trial, patients must have had a confirmed genetic diagnosis of WHIM syndrome, be at least 18 years of age and have a neutrophil count equal to or less than 400/μL or a lymphocyte count equal to or less than 650/μL. Patients who had been infected with the human immunodeficiency virus, or HIV, were excluded from the trial, as were patients with recent exposure to plerixafor.

In the trial, patients received escalating doses of mavorixafor starting at 50 mg once daily to 400 mg once daily. Patients received starting doses higher than 50 mg once daily as the trial progressed based on the safety and biomarker response data of earlier patients enrolled in the trial. Patients were dose-escalated from their starting dose based on an in-hospital 24-hour measurement of ANCs and ALCs above or below the pre-defined target thresholds of 600/μL and 1,000/μL, respectively.

Neutrophil counts of less than 500/μL are associated with increased risk of infection and are classified as grade 4 (severe or life threatening) by the National Cancer Institute. The study's entry criterion was set at a neutrophil count equal to or less than 400/μL, to assure that patients in the trial had neutrophil counts in this "severe or life threatening" category. The response threshold was set at 600/μL to reflect a minimum 50% increase in neutrophil counts and meaningfully above the critical grade 4 limit.

In contrast to neutrophil counts, there is no clinical correlate established for degrees of lymphopenia, so the entry criterion for the trial was set to be in the middle of grade 2 severity (moderate lymphopenia). The response threshold of 1000/μL is within the normal range for lymphocytes and represents a minimum of 50% improvement over lymphocytes counts at trial entry. Given lymphocytes play a key role in the immune response and lymphopenia is a characteristic of WHIM syndrome, a return to normal lymphocyte counts is expected also to be associated with improved immune function.

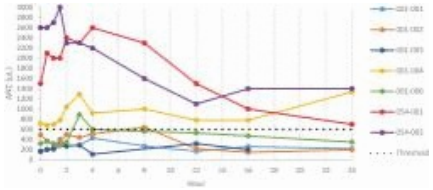
To assess the blood levels of ANCs and ALCs, patients received mavorixafor at the same dose for at least three weeks to reach steady state. Patients then completed a 24-hour hospital stay at the beginning of which patients were given the appropriate dose of mavorixafor. We then took blood measurements for ANC and ALC over the 24-hour period through standard complete blood count, or CBC, methods. The main biomarker endpoint of the trial was a measurement of the amount of time and by how much ANCs and ALCs remained above or below the predefined thresholds over the 24-hour period. The treatment goal for this endpoint was to increase counts to 600/μL for neutrophils and 1000/μL for lymphocytes.

We completed the dose-titration portion of the Phase 2 trial in March 2018 and, based on the reported results, the Data Review Committee, or DRC, recommended the Phase 3 dose of 400 mg administered once daily based on these results. Following completion of the dose-titration portion of the Phase 2 trial, patients were allowed to continue on study drug in a Phase 2 open-label extension trial. Five patients continue to receive mavorixafor in the open-label extension trial.

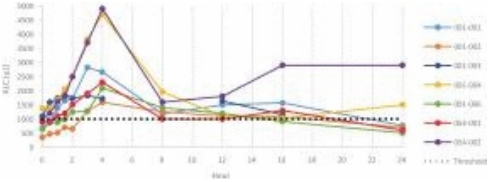
In the trial, mavorixafor was reported to be well tolerated across all doses. Treatment-related adverse events reported were dry mouth (n=2), nausea (n=2), dry eye (n=1), nasal dryness (n=1), dyspepsia (n=1), conjunctivitis (n=1) and rash (n=1). No serious adverse events were reported that were deemed related to treatment with mavorixafor and no adverse event met criteria for a treatment-limiting toxicity. One patient discontinued treatment after two weeks because of a treatment-related Grade 1 rash and is not included in the evaluable patients discussed below. Two patients completed the six months of dose titration and declined to continue on treatment in the open-label extension.

In the trial, for all evaluable patients, the lymphocyte counts exceeded threshold at doses at or above 50 mg per day and, except for one patient, the neutrophil counts exceeded threshold at doses at or above 300 mg per day. In the graphs below, the 24-hour in-hospital ANCs and ALCs of all seven patients dosed at 300 mg per day are shown. The ANC of three of the seven patients exceeded the 600/μL neutrophil threshold and therefore were not further dose-escalated. Of the patients whose neutrophil levels did not exceed the target thresholds, three were then dose escalated to 400 mg per day, and one patient declined to continue in the trial. The ALC for six patients (6/7) exceeded the 1,000/μL lymphocyte threshold at the 300 mg dose.

ANC Levels All Patients at 300 mg

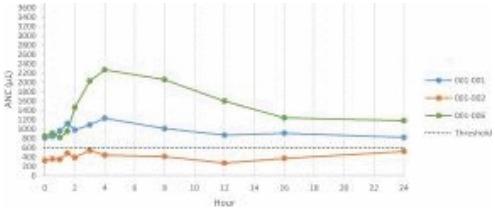


ALC Levels All Patients at 300 mg

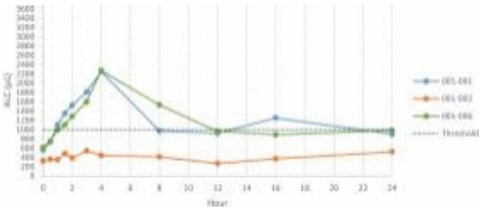


In the graphs below, the 24-hour in-hospital ANCs and ALCs of three patients who were dose-escalated to 400 mg per day are shown. Two of three patients achieved counts above the threshold levels of both neutrophils and lymphocytes. Although the third patient did not achieve neutrophil counts above threshold, this patient has reported no infections during the nine months since initiating treatment at 400 mg per day, which is reported to be meaningfully reduced as compared to her prior history reported by the trial investigator. Among the three patients with a combined 27 months of treatment at the 400 mg dose, only two infections have been reported at that dose. We believe this may be indicative of the potential clinical benefit of daily dosing with mavorixafor despite varying degrees of increases in the levels of ANCs and ALCs.

ANC Levels All Patients at 400 mg



ALC Levels All Patients at 400 mg



In summary, at mavorixafor doses of 300 mg and 400 mg administered once daily, we observed that five of seven patients achieved the pre-defined thresholds for neutrophils and six of seven patients achieved the pre-defined thresholds for lymphocytes. Doses of mavorixafor above 400 mg per day were not tested in the Phase 2 trial due to the meaningful increased levels of ANCs and ALCs and the favorable tolerability profile observed with the 400 mg per day dose. Based on these data, the DRC recommended that the 400 mg daily dose of mavorixafor be utilized in the Phase 3 pivotal clinical trial.

In addition to the favorable tolerability profile and the achievement of threshold levels of ANCs and ALCs, we observed preliminary evidence of clinical activity in the form of reductions in wart lesions and physician-reported reductions in infection rates. Prior to entering the trial, one patient was reported to have a long history of severe wart lesions that were refractory to all available treatments. This patient had a dramatic and visible reduction in wart burden observed at 26 weeks of treatment; wart burden continued to decrease after 55 weeks of treatment with mavorixafor. The patient has had no treatment of any kind during the trial for warts and the reductions in wart lesions was reported by the investigator as a probable drug effect of mavorixafor. This patient continues to be treated in the extension arm of this trial.

Our Planned Phase 3 Clinical Trial for Patients with WHIM Syndrome

We intend to initiate the Phase 3 pivotal clinical trial in WHIM syndrome in the second quarter of 2019. We expect that the Phase 3 pivotal clinical trial will enroll a minimum of 18 and up to a maximum of 28 WHIM patients across sites in the United States and globally. To be included in the Phase 3 pivotal clinical trial, patients must have had a genetically confirmed diagnosis of WHIM syndrome, have a neutrophil count equal to or less than 400/μL, and a lymphocyte count equal to or less than 650/μL, and no prior exposure to plerixafor. These inclusion criteria are the same as the inclusion criteria used in the Phase 2 trial with respect to these parameters. We intend to randomize patients on a one-to-one basis into the mavorixafor and placebo arms and to administer the study drug at a dose of 400 mg per day over a 52-week treatment period. As reviewed with the FDA, the primary endpoint will be biomarker of neutrophil count time above threshold, or TAT, where the threshold is defined as 500 cells/μL. We plan to also measure other secondary endpoints, including the difference between the treatment arm and the placebo arm in the lymphocyte counts above the predefined 1,000/μL threshold, frequency and severity of infections, number and severity of warts, antibody levels following revaccination, Ig levels, frequency of events requiring rescue therapy, hospitalizations, quality of life metrics, and Patient Reported Outcomes, or PROs, to further assess the potential clinical benefit of mavorixafor in WHIM patients. The Phase 3 pivotal clinical trial's secondary endpoints, including infection rates and wart burden assessments, and secondary endpoint hierarchy were also reviewed with the FDA. We will enroll patients ages 12 years and older will receive 400 mg, once daily, of mavorixafor. Following completion of the Phase 3 pivotal clinical trial, patients will be allowed to continue on study drug in an open-label expansion trial. If the Phase 3 pivotal clinical trial is successful, we believe this trial will be the only trial required to submit a New Drug Application, or NDA, filing in the United States.

We have participated in an initial Scientific Advice interaction with the EMA. Based on EMA feedback, we believe that a biomarker endpoint similar to that assessed in the Phase 2 trial will be acceptable for the primary endpoint of the Phase 3 pivotal clinical trial to support approval in the European Union. Additionally, we have submitted a request for Orphan Drug Designation, or ODD, in Europe; Orphan Drug Designation was granted for mavorixafor for the treatment of WHIM syndrome in the United States by the FDA.

Mavorixafor in SCN and Additional PIs

We believe mavorixafor may be used to treat a number of additional PIs beyond WHIM syndrome. Particularly, we believe mavorixafor can potentially treat patients with severe congenital neutropenia, or SCN. Like WHIM syndrome, SCN is a rare blood disorder similarly characterized by increased risks of infections and cancer due to abnormally low levels of certain white blood cells, including neutrophils and lymphocytes, in the body. Additionally, some sub-types of SCN have mechanisms that overlap with signaling of the CXCL12/CXCR4 pathway. G-CSF is the standard of care for SCN and is used to stimulate the bone marrow to produce neutrophils. Side effects of G-CSF include disabling bone pain, which can be more severe in certain age groups. Additional, less common, treatment-limiting complications of chronic G-CSF administration include myelofibrosis and leukemia. In SCN cases that are unresponsive to G-CSF, or if leukemia has developed, bone marrow transplants have been made with varying degrees of success. Bone marrow transplants bring additional risks into the management of the disorder. We plan to initiate a Phase 1 clinical trial of mavorixafor in 2019 in certain genetically defined SCNs to assess the potential to expand the use of mavorixafor into additional PIs beyond WHIM syndrome. Given the data that our trials have generated to date in WHIM patients, we expect to start dosing patients with 400 mg of mavorixafor once daily.

Mavorixafor in Waldenström macroglobulinemia

We believe mavorixafor may be used to treat certain blood cancers, including Waldenström macroglobulinemia, or WM. WM, is a rare form of non-Hodgkin's lymphoma and B-cell lymphoproliferative disorder. The WM landscape has recently been revolutionized by whole-genome sequencing that has identified genetic mutations in the disease. Approximately 30-40% of WM patients have been shown to have gain-of-function, WHIM syndrome-like mutations in the CXCR4 gene in the cancer cells that define this rare form of lymphoma. In WM, somatic mutations of CXCR4 have been found to be associated with activating and pro-survival signaling of tumor cells, as well as the possible acquisition of resistance to several drugs, including anti-CD20 monoclonal antibody, and BTK inhibitors, such as ibrutinib, the current standard of care. For example, in WM patients who have been treated with ibrutinib, patients with WHIM syndrome-like mutations have generally not responded as well to treatment

as compared to patients without the somatic WHIM syndrome mutation. The mPFS for WM patients treated with ibrutinib with WHIM-like mutations has been shown to be approximately two years, whereas patients without the mutation have an mPFS of well over five years. We plan to initiate a Phase 1/2 clinical trial of mavorixafor in WM in 2019.

X4P-002

We are also developing X4P-002, a CXCR4 antagonist that has unique properties that we believe will enable it to penetrate the blood-brain barrier with a potential to provide therapeutic exposures necessary to treat glioblastoma multiforme, or GBM. GBM is the one of the most aggressive forms of brain cancer. GBM accounts for about 15% of brain cancers and it is estimated that there were 12,000 new cases of GBM in the United States in 2016. The five-year overall survival is 10%, which demonstrates the need for new therapies that effectively treat GBM.

Malignancies of the brain present greater demands on drug distribution within the body than other tumors due to the blood-brain barrier that actively transports undesired molecules out of the brain. We believe that X4P-002 is the only CXCR4 antagonist product candidate that has been shown, in preclinical studies, to penetrate the blood-brain barrier in levels that exceed the targeted levels required for an optimized anti-cancer effect in animal studies. As a result, we believe that X4P-002 has the potential to be a first-in-class treatment for GBM.

X4P-003

We are also developing X4P-003, a second generation molecule for the treatment of rare diseases linked to defects in CXCR4 trafficking. X4P-003 is designed to have an enhanced pharmacokinetic/pharmacodynamic profile relative to mavorixafor, which could allow X4P-003 to deliver improved patient compliance and ease of use to better serve patients suffering from chronic rare diseases.

Our Immuno-Oncology Programs

Overexpression of CXCL12, the ligand for CXCR4, is found in over 20 solid and blood-derived tumor types, indicating a key role of the CXCL12/CXCR4 pathway in pro-tumor signaling and immunosuppression. We have completed two pilot open label Phase 1b clinical trials in immuno-oncology: a Phase 1b clinical trial of mavorixafor in combination with pembrolizumab to assess our pharmacodynamics in patients with advanced melanoma alone and a Phase 1b clinical trial of mavorixafor in combination with nivolumab for the treatment of patients with clear cell renal cell carcinoma, or ccRCC, after patients have become refractory to nivolumab. Data from our completed Phase 1b clinical trial in melanoma support single-agent activity in the TME with meaningful increases in activated CD8+ T-cells observed. In the Phase 1b trial in ccRCC, we observed clinical improvements in a majority of the patients. We observed a favorable tolerability profile across both trials. We are currently conducting the Phase 2a portion of the open-label Phase 1/2 clinical trial in ccRCC in combination with axitinib. In the Phase 1b portion of the trial, mavorixafor exhibited a favorable tolerability profile, we determined the maximum tolerated dose and patients showed early signs of clinical activity, including a complete response in one heavily pre-treated patient. In June 2018, we reported preliminary results from 47 evaluable patients in the Phase 2a portion of this trial in ccRCC with axitinib, in which we observed a favorable tolerability profile, as well as promising signs of clinical activity in heavily pretreated patients with ccRCC. We expect to announce progression-free survival, or PFS, data as part of an anticipated abstract to be submitted for presentation at a major medical conference in the second half of 2019. We intend to pursue a strategic collaboration for future development and potential commercialization of mavorixafor in ccRCC and other potential immuno-oncology indications.

Our Ongoing Phase 1/2 Clinical Trial in Combination with Axitinib in ccRCC

In 2015, we initiated a two-part Phase 1/2 clinical trial of mavorixafor for the treatment of patients with ccRCC who had received at least one prior line of therapy across multiple sites in the United States and South Korea. We are currently assessing mavorixafor in the Phase 2a portion of the open-label Phase 1/2 trial. The Phase 1b portion of the trial was an open-label, three-by-three dose escalation trial. The goals of the trial are to evaluate the safety, pharmacokinetics and anti-tumor activity of mavorixafor in combination with axitinib to confirm the dose to

be used in future clinical development. All patients in the Phase 1b portion of the trial were administered once-daily mavorixafor and 5 mg of axitinib twice daily. Tumor lesions were assessed by computerized tomography, or CT, scans every eight weeks and assessed by central review. Additionally, blood draws to assess drug levels, CBCs and other biomarkers were collected throughout the trial.

Efficacy is being evaluated based on the industry standard Response Evaluation Criteria in Solid Tumors, or RECIST, which are the unified response assessment criteria agreed to by the World Health Organization, United States National Cancer Institute, and European Organisation for Research and Treatment of Cancer. RECIST defines disease progression and tumor response based on these international standards.

We enrolled 16 patients in the Phase 1b portion of the trial and 14 were evaluable for tumor response. We observed an objective response rate, or ORR, of 28.6%, with three partial responses, or PRs, and one complete response, or CR. Additionally, there were nine stable diseases, SDs, and one progressive diseases, or PD, which resulted in a disease control rate, or DCR, of 92.9%. Mavorixafor doses of 200 mg twice per day, 400 mg once per day, and 600 mg once per day were tested in the Phase 1b portion of the trial, and 400 mg once per day was chosen for the open label Phase 2a portion of the trial.

In June 2018, we presented preliminary results from 47 evaluable patients from the ongoing Phase 2a portion of the trial. Patients in this portion of the trial were heavily pre-treated, with 75% of patients having had two or more prior treatments. The interim observed ORR was 23%, with 10 (21%) PRs and one (2%) CR. Additionally, there were 27 SDs and nine PDs, which resulted in a DCR of approximately 81% for this interim analysis.

Best Response* in Clinically Evaluable Patients (N = 47)**

Complete Response (CR)	**1 (2%)**
Partial Response (PR)	**10 (21%)**
Stable Disease (SD)	**27 (57%)**
Progressive Disease (PD)	**9 (19%)**
Objective Response Rate (CR + PR)	**23%**

Clinical cut-off date: March 23, 2018

* Based on RECIST 1.1 criteria
** Response data from central review is currently pending for remaining 18 patients

Safety data, which was reported in our American Society of Clinical Oncology Meeting presentation in June 2018, showed that mavorixafor in combination with axitinib was reported to be well tolerated. Treatment-related serious adverse events were diarrhea, hyperkalemia, and hypertension (two patients each, 3%), and blood creatinine increased, nausea, sepsis and trachea-esophogeal fistula (one patient each, 1.5%). The observed tolerability profile of the combination was consistent with axitinib single-agent adverse events observed in other clinical trials.

We intend to present updated data from the Phase 2a portion of the trial in the second half of 2019 and intend to include additional clinical metrics of potential activity, such as mPFS and duration of response as well as additional safety information.

Our Phase 1b Clinical Trial in Combination with Nivolumab in ccRCC

We have completed a Phase 1b trial of mavorixafor for the treatment of patients with ccRCC in combination with the approved immuno-oncology therapy, nivolumab, a PD-1 checkpoint inhibitor. The primary objective of the trial was to evaluate the safety and tolerability of mavorixafor in combination with nivolumab. The trial enrolled patients who have not responded to nivolumab, but who were maintained on nivolumab while mavorixafor was added to their treatment regimen. In addition to safety and tolerability, the trial evaluated early signs of biological activity using biomarkers, and clinical activity as measured by ORR.

Enrolled patients received 400 mg of mavorixafor once daily and continued to receive standard bi-weekly nivolumab therapy. Median duration of treatment with the combination was 3.7 months (range one to 15 months). We observed that five of nine patients had clinical improvements in tumor shrinkage with the addition of mavorixafor.

In the trial, we observed that mavorixafor in combination with nivolumab had an acceptable tolerability profile in ccRCC patients. The most frequent drug related adverse events were diarrhea, nasal congestion, ALT/AST increase, dry eye and fatigue. No grade 4 or 5 adverse events occurred. All Grade 3 serious adverse events related to the combination treatment were reported to be manageable with appropriate intervention. Two patients experienced serious adverse events: one had mucosal inflammation and rash maculo-papular and another had an ALT/AST increase and autoimmune hepatitis.

In addition, in the trial, combination therapy with mavorixafor and nivolumab exhibited anti-tumor activity in some patients with advanced ccRCC who were previously unresponsive to nivolumab monotherapy. Four patients who had progressed on prior nivolumab monotherapy we observed to have a best response of stable disease with the additional mavorixafor to nivolumab treatment. Of the five patients who were stable on prior nivolumab monotherapy, one had a partial response with combination therapy of mavorixafor and nivolumab. Serum biomarker analyses identified significant early changes in cytokines and chemokines, including CXCL9, a chemoattractant ligand for cytotoxic T-cell migration.

Our Phase 1b Clinical Biomarker Trial in Advanced Melanoma

We have completed a Phase 1b biomarker clinical trial in 16 patients with Stage III and IV melanoma. This multi-center trial evaluated the safety and tolerability of mavorixafor alone and in combination with the immuno-oncology therapy pembrolizumab, an approved PD-1 checkpoint inhibitor. The trial evaluated the immune profile of participants' tumor biopsies and blood to assess changes of key immune cell profiles and inflammatory response markers. Nine patients had both baseline (pre-dose) and post-mavorixafor treatment-evaluable biopsies and were considered as evaluable patients to be included in the analysis. Results from the tumor biopsies taken from melanoma patients, before and after receiving single agent mavorixafor treatment for three weeks, were analyzed. Analyses showed three weeks of single agent mavorixafor monotherapy was associated with tumor immunity. Enhanced immunity was indicated by:

- increased proliferating CD8+ cells, indicative of cytotoxic T-cell activation;
- increased IFN-gamma gene expression signature score, suggesting enhanced antigen priming and activation;
- increased Tumor Inflammation Signature, or TIS, indicative of increased inflammation status in the TME;
- increased CD8+ T-cell density at the tumor interface, with the total density of CD8+ cells inside the tumor boundary area increased four-fold compared with baseline;
- increased numbers of cells expressing CD3 antigens, a pan T-cell marker, within tumor borders, and decreased expression of VISTA, a checkpoint molecule that inhibits T-cell activation and proliferation; and/or
- increases in multiple chemoattractant factors in serum, consistent with increased trafficking of immune cells post CXCR4 inhibition.

After single agent mavorixafor treatment, patients received mavorixafor in combination with pembrolizumab for an additional six weeks. Continued signs of positive immune cell changes in the TME were seen with combination treatment. Treatment of additional patients in the trial showed that mavorixafor as a single agent, and in combination with pembrolizumab, continued to be well tolerated. Treatment-related adverse events were diarrhea, fatigue, rash macro-papular and dry eye.

Arsanis Programs

We are currently undertaking a strategic review of our development programs focused on applying monocloncal antibody, or mAb, immunotherapies to address serious infectious diseases that were being developed by Arsanis prior to the Merger. As part of this review, we intend to explore potential collaborations, out-licensing or sale opportunities with respect to, or the discontinuation of, our ASN100 *Staphylococcus aureus pneumonia* and ASN500 respiratory syncytial virus, or RSV, programs. In addition, we intend to enter into discussions with Bravo Biosciences, LLC, or Bravos, to reduce our already minimal ongoing support of their development of ASN300 for *Klebsiella pneumonia* or ASN200 for *Escherichia coli*, which are the subject of an option and license agreement executed with subsidiaries of Bravos during the first half of 2018.

Competition

The pharmaceutical and biotechnology industries are characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. We face potential competition from many different sources, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions, governmental agencies and public and private research institutions. Any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future.

Many of our competitors may 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. Other firms also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient enrollment for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Mergers and acquisitions in the pharmaceutical, biotechnology and diagnostic industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors with us, particularly through collaborative arrangements with large and established companies.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize therapeutics that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain marketing approvals for their products more rapidly than we may obtain approval for our products, which could result in our competitors establishing a strong market position before we are able to enter the market. In addition, our ability to compete may be affected because in some cases insurers or other third-party payors, including government programs, seek to encourage the use of generic products. This may have the effect of making branded products less attractive, from a cost perspective, to buyers.

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 WM, the Dana Farber Cancer Institute has initiated a trial to study the BMS CXCR4 antibody (IV infusion) in the treatment of WM patients with CXCR4 mutations.

In WM, 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. Our principal competitors in ccRCC include Pfizer, Novartis, BMS, and Merck. In glioblastoma, our principal competitors include Genentech/Roche and BMS.

Manufacturing

We do not own or operate, and currently have no plans to establish, manufacturing facilities for the production of clinical or commercial quantities of mavorixafor or any of our other product candidates. We currently rely, and expect to continue to rely, on third parties for the manufacture of our product candidates and any products that we may develop.

We currently engage a single third-party manufacturer to provide the active pharmaceutical ingredient, or API, for mavorixafor. We also engage a single third-party manufacturer to provide fill and finish services for the final drug product formulation of mavorixafor for use in our clinical trials.

We obtain the supplies of our API and drug products from these manufacturers pursuant to typical industry standard clinical supply agreements. We believe that both API and drug product manufacturers have the capability and capacity to manufacture currently projected clinical trial supply and commercial volumes of mavorixafor and we are engaged in active discussions with both parties to plan commercial manufacturing arrangements. We obtain the supplies of our product candidates from these manufacturers under master services contracts and specific work orders. However, we do not have long-term supply arrangements in place. We do not currently have arrangements in place for redundant supply or a second source for API for mavorixafor. If any of our current manufacturers becomes unavailable to us for any reason, we believe that there are a number of potential replacements, although we might incur some delay in identifying and qualifying such replacements. We intend to identify and qualify additional manufacturers to provide bulk drug substance and drug product services prior to submission of a new drug application, or NDA, to the FDA, if necessary, to ensure sufficient commercial quantities of mavorixafor.

License Agreements

License Agreement with Genzyme

In July 2014, we entered into a license agreement with Genzyme pursuant to which we were granted an exclusive license to certain patent applications and other intellectual property owned or controlled by Genzyme related to the CXCR4 receptor to develop and commercialize products containing licensed compounds (including but not limited to mavorixafor) for all therapeutic, prophylactic and diagnostic uses with the exception of autologous and allogenic human stem cell therapy. Genzyme has retained the exclusive right to use the intellectual property licensed to us in specific indications related to Genzyme's product Mozobil® and allogenic/autologous hematopoietic stem cell transplantation treatments. Genzyme has also retained the non-exclusive right to conduct preclinical research involving compounds in any field, including any fields licensed to us, but has not retained rights to conduct any clinical development or commercialization of those compounds identified in the agreement in any of the fields licensed to us. We are primarily responsible for the preparation, filing, prosecution and maintenance of all patent applications and patents covering the intellectual property licensed to us under the agreement at our sole expense.

Under the terms of the agreement, we are obligated to use commercially reasonable efforts to develop and commercialize licensed products for use in the field in the United States and at least one other major market country. We have the right to grant sublicenses of the licensed rights that cover mavorixafor to third parties. If we wish to grant a sublicense to any licensed product other than mavorixafor, we are obligated to first offer the sublicense to Genzyme. If Genzyme expresses written interest for the sublicense, then we will negotiate exclusively with Genzyme for a certain stated period to obtain a license to such rights, after which Genzyme shall have no further rights with respect to such licensed product and we will be free to negotiate a sublicense with respect to such licensed product with any third party.

We paid Genzyme an initial fee on the effective date of the agreement and an additional fee in the amount of $300,000 upon the satisfaction of X4's financing obligation by X4's Series A round financing.

X4 issued Genzyme 1,129,823 shares of its common stock following its Series A financing, equal to 10% of its outstanding common stock at the time following the Series A financing. No further issuance of shares is required under the terms of the license.

We are obligated to pay Genzyme milestone payments in the aggregate amount of up to $25,000,000, contingent upon our achievement of certain late-stage regulatory and sales milestones with respect to licensed products. In addition, X4 was required to make a one-time milestone payment to Genzyme upon the consummation by X4 of a change of control transaction, in an amount equal to 5.5% of the consideration paid to its equity holders, other than Genzyme, in connection with such change of control transaction, after deducting its outstanding debt obligations and the aggregate cash investments made by X4's equity holders prior to the closing of the change of control transaction. The Merger qualified as a change of control event, as defined in the license agreement, but resulted in no payment being due to Genzyme under the license agreement.

We are obligated under the agreement to pay Genzyme tiered royalties based on net sales of licensed products that we commercialize under the agreement. Our obligation to pay royalties for each licensed product expires on a country-by-country basis on the latest of (i) the expiration of licensed patent rights that cover that licensed product in that country, (ii) the expiration of regulatory exclusivity in that country and (iii) ten years after the first commercial sale of such licensed product in that country. Royalty rates are subject to reduction under the agreement in specified circumstances, including in any country if we are required to obtain a license from any third party to the extent our patent rights might infringe the third party's patent rights, if a licensed product is not covered by a valid claim in that country or if sales of generic products reach certain thresholds in that country. If we enter into a sublicense under the agreement, we will be obligated to pay Genzyme a percentage of certain upfront, maintenance fees, milestone payments and royalty payments paid to us by the sublicensee.

The term of the agreement will continue until the later of the expiration of the last to expire valid claim of the patents licensed under the agreement that cover any licensed product, the expiration of regulatory exclusivity applicable to any licensed product and 10 years from the date of first commercial sale of any licensed product. Either we or Genzyme may terminate the agreement in the event of the bankruptcy or uncured material breach by the other party. Genzyme may terminate the agreement if we or our affiliates initiate a patent challenge of the patents licensed under the agreement. We may terminate the agreement immediately upon notice to Genzyme if we reasonably believe that the development or commercialization of a licensed compound or product under the agreement would result in a material safety issue for patients.

License Agreement with Georgetown University

In December 2016, we entered into a license agreement with the Georgetown University, or Georgetown, pursuant to which we obtained an exclusive, worldwide license to practice certain methods, and to make, have made, use, sell, offer for sale and import products, covered by licensed patent rights co-owned by Georgetown. The rights licensed to us are for all therapeutic, prophylactic and diagnostic uses in all disease indications in humans and animals. We have the right to grant sublicenses of the licensed rights to third parties to the extent consistent with the terms of the agreement.

Under the terms of the agreement we paid a one-time only, upfront fee of $50,000, and we may be required to pay milestone payments of up to an aggregate of $800,000 related to commercial sales of a licensed product. We are responsible for all patent prosecution costs incurred with respect to the licensed patents. We are obligated under the agreement to use commercially reasonable efforts to develop and commercialize licensed product, to make licensed product reasonably available to the public, to obtain government approvals for licensed product and to market licensed product in quantities sufficient to meet the market demand.

The term of the license agreement will continue until the expiration of the last valid claim within the patent rights covering the licensed products. Georgetown may terminate the agreement or convert our license to non-exclusive in the event (i) we fail to pay any amount and fail to cure such failure within 30 days after receipt of notice, (ii) we default in our obligation to obtain and maintain insurance and fail to remedy such breach within 45 days after receipt of notice, (iii) we declare insolvency or bankruptcy or (iv) we materially default in the performance of any material obligations under the agreement which is not cured within a certain period from the date of written notice of such default. We may terminate the agreement at any time upon at least 60 days' written notice.

License Agreement with Beth Israel Deaconess Medical Center

In December 2016, we entered into a license agreement with Beth Israel Deaconess Medical Center, or BIDMC, pursuant to which we obtained an exclusive, worldwide license to make, have made, use, sell, offer for sale and import of licensed products and certain processes covered by licensed patent rights co-owned by BIDMC and a nonexclusive royalty-free right to use certain information pertaining to any invention claimed in the licensed patents that is owned by BIDMC to develop, make, have made, use, have used, sell, have sold and commercialize such licensed products and processes. The rights licensed to us are for all fields of use. We have the right to grant sublicenses of the licensed rights to third parties to the extent consistent with the terms of the agreement.

Under the terms of the agreement we paid a one-time only, upfront fee of $20,000 and we are responsible for all future patent prosecution costs.

The term of the license agreement will continue until the expiration of the last valid claim within the patent rights covering the licensed product. BIDMC may terminate the agreement in the event (i) we fail to pay any amount and fail to cure such failure within 15 days after receipt of notice, (ii) the insurance coverage that we are obligated to maintain under the agreement is terminated and we fail to obtain replacement insurance within a certain period of time following notice to BIDMC, or (iii) we declare insolvency or bankruptcy. In addition, if we are in material breach of any material provisions of the agreement and fail to remedy such breach within 60 days after receipt of notice, BIDMC may terminate the agreement or terminate any licenses granted under the agreement with respect to the country or countries in which such material breach has occurred. We may terminate the agreement at any time upon at least 90 days' written notice.

Intellectual Property

Our ability to commercialize our product candidates depends in large part on our ability to obtain and maintain intellectual property protection for our product candidates, including mavorixafor, and our preclinical compounds and core technologies. Our policy is to seek to protect our intellectual property position by, among other methods, filing U.S. and foreign patent applications related to the technology, inventions and improvements that are important to the development and implementation of our business strategy. We also rely on trade secrets, know-how and continuing technological innovation to develop and maintain our proprietary position.

We file patent applications directed to our product candidates, preclinical compounds and related technologies to establish intellectual property positions on these compounds and their uses in disease. As of March 15, 2019, we owned or exclusively licensed 12 issued U.S. patents, 10 pending U.S. non-provisional patent applications, five pending U.S. provisional patent applications, and approximately 120 PCT and foreign patents and patent applications in the following foreign jurisdictions: Belgium, Brazil, Canada, China, European Patent Office, France, Germany, Great Britain, Hong Kong, India, Ireland, Italy, Israel, Japan, Lichtenstein, Mexico, Netherlands, Spain, Sweden and Switzerland.

As of March 15, 2019, our in-licensed intellectual property portfolio for mavorixafor included one issued U.S. patent and one allowed U.S. patent application directed to compositions of matter for mavorixafor, which is expected to expire in December 2022 excluding possible patent term extensions of up to an additional five years. The intellectual property portfolio for mavorixafor also included one issued U.S. patent with claims directed to a crystalline salt form of mavorixafor, one issued U.S. patent directed to pharmaceutical compositions of mavorixafor in unit dosage form, and four issued U.S. patents directed to methods of making mavorixafor and key intermediates. We also had four issued U.S. patents directed to compositions and methods of making chemical compounds related to the X4P-001 program. Approximately 85 corresponding PCT and foreign patents and patent applications directed to compositions of matter and related chemical compounds as well as methods of making and methods of use were issued or pending. All of the above patents and patent applications were exclusively licensed to us pursuant to the terms of the Genzyme license agreement.

Additionally, we have filed our own patent applications with respect to the mavorixafor and X4P-002 product candidates. Some of these patent applications are co-owned with Genzyme, BIDMC, or Georgetown, with their rights exclusively licensed to X4. As of March 15, 2019, our independently generated intellectual property portfolio included six pending U.S. non-provisional patent applications, five pending U.S. provisional patent applications, and approximately 22 pending PCT and foreign patent applications related to our mavorixafor clinical programs in cancer and primary immunodeficiencies; and three pending U.S. non-provisional patent applications and 13 pending PCT and foreign patent applications related to our preclinical compounds and X4P-002 in glioblastoma. Patents issuing from these applications, if any, are expected to expire between 2036 and 2039.

The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries, including the United States, the patent term is 20 years from the earliest filing date of a non-provisional patent application. In the United States, a patent's term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the U.S. Patent and Trademark Office, or the USPTO, in examining and granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier filed patent. The term of a U.S. patent that covers a drug or biological product may also be eligible for patent term extension when approval from the FDA is granted, provided statutory and regulatory requirements are met. In the future, if our product candidates receive approval from the FDA or foreign regulatory authorities, we expect to apply for patent term extensions on issued patents covering those products, depending upon the length of the clinical trials for each drug and other factors. There can be no assurance that any of our pending patent applications will issue or that we will benefit from any patent term extension or other favorable adjustment to the term of any of our patents.

As with other biotechnology and pharmaceutical companies, our ability to maintain and solidify our proprietary and intellectual property position for our product candidates, including mavorixafor, and its preclinical compounds, and our core technologies will depend on our success in obtaining effective patent claims and enforcing those claims if granted. However, patent applications that we may file or license from third parties may not result in the issuance of patents. We also cannot predict the breadth of claims that may be allowed or enforced in our patents. Any issued patents that we may receive in the future may be challenged, invalidated or circumvented. For example, prior to March 16, 2013, in the United States, patent applications were subject to a "first to invent" rule of law. Applications filed after March 16, 2013 (except for certain applications claiming the benefit of earlier-filed applications) are subject to a "first to file" rule of law.

Discoveries reported in the scientific literature often lag 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. We cannot be certain that any existing or future application will be subject to the "first to file" or "first to invent" rule of law, that we were the first to make the inventions claimed in our existing patents or pending patent applications subject to the prior laws, or that we were the first to file for patent protection of such inventions subject to the new laws. If third parties prepare and file patent applications in the United States that also claim technology we have claimed in our patents or patent applications, we may have to participate in interference proceedings in the USPTO to determine priority of invention, which could result in substantial costs to us, even if the eventual outcome is favorable to us. In addition, because of the extensive time required for clinical development and regulatory review of a product candidate we may develop, it is possible that, before any of our product candidates can be commercialized, any related patent may expire or remain in force for only a short period following commercialization, thereby reducing any advantage of any such patent.

In addition to patents, we rely upon unpatented trade secrets, know-how, and continuing technological innovation to develop and maintain our competitive position. We seek to protect our proprietary information, in part, by using confidentiality agreements with our collaborators, scientific advisors, employees and consultants, and invention assignment agreements with our employees. We also have agreements requiring assignment of inventions with selected consultants, scientific advisors and collaborators. The confidentiality agreements are designed to protect our proprietary information and, in the case of agreements or clauses requiring invention assignment, to grant us ownership of technologies that are developed under those agreements.

Government Regulation and Product Approval

The FDA Approval Process

In the United States, pharmaceutical products are subject to extensive regulation by the FDA. The Federal Food, Drug, and Cosmetic Act, or the FDCA, and other federal and state statutes and regulations, govern, among other things, the research, development, testing, manufacture, storage, recordkeeping, approval, labeling, promotion and marketing, distribution, post-approval monitoring and reporting, sampling, and import and export of pharmaceutical products. Failure to comply with applicable U.S. requirements may subject a company to a variety of administrative or judicial sanctions, such as imposition of clinical holds, refusal by the FDA to approve pending NDAs, warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement, civil penalties and criminal prosecution.

Pharmaceutical product development in the United States typically involves preclinical or other nonclinical laboratory and animal tests and the submission to the FDA of an IND, which must become effective before clinical testing may commence. For commercial approval, the sponsor must submit adequate tests by all methods reasonably applicable to show that the drug is safe for use under the conditions prescribed, recommended or suggested in the proposed labeling. The sponsor must also submit substantial evidence, generally consisting of adequate, well-controlled clinical trials to establish that the drug will have the effect it purports or is represented to have under the conditions of use prescribed, recommended or suggested in the proposed labeling. In certain cases, the FDA may determine that a drug is effective based on one clinical study plus confirmatory evidence. Satisfaction of the FDA pre-market approval requirements typically takes many years and the actual time required may vary substantially based upon the type, complexity and novelty of the product or disease.

Nonclinical tests include laboratory evaluation of product chemistry, formulation and toxicity, as well as animal studies to assess the characteristics and potential safety and efficacy of the product. The conduct of the nonclinical tests must comply with federal requirements, including the FDA's good laboratory practices regulations and the U.S. Department of Agriculture's, or USDA's, regulations implementing the Animal Welfare Act. The results of nonclinical testing are submitted to the FDA as part of an IND along with other information, including information about product chemistry, manufacturing and controls, and a proposed clinical trial protocol. Long-term nonclinical tests, such as animal studies of reproductive toxicity and carcinogenicity, may continue after the IND is submitted.

A 30-day waiting period after the submission of each IND is required prior to the commencement of clinical testing in humans. If the FDA has not imposed a clinical hold on the IND or otherwise commented or questioned the IND within this 30-day period, the clinical trial proposed in the IND may begin.

Clinical trials involve the administration of the investigational new drug to healthy volunteers or patients under the supervision of a qualified investigator. Clinical trials must be conducted: (i) in compliance with federal regulations, (ii) in compliance with GCP, an international standard meant to protect the rights and health of patients and to define the roles of clinical trial sponsors, administrators and monitors, and (iii) under protocols detailing the objectives of the trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. Each protocol involving testing on U.S. patients and subsequent protocol amendments must be submitted to the FDA as part of the IND.

The FDA may order the temporary, or permanent, discontinuation of a clinical trial at any time or impose other sanctions if it believes that the clinical trial either is not being conducted in accordance with the FDA requirements or presents an unacceptable risk to the clinical trial patients. The trial protocol and informed consent information for patients in clinical trials must also be submitted to an institutional review board, or IRB, at each site where a trial will be conducted for approval. An IRB may also require the clinical trial at the site to be halted, either temporarily or permanently, for failure to comply with the IRB's requirements or may impose other conditions.

Clinical trials to support NDAs for marketing approval are typically conducted in three sequential phases, but the phases may overlap. In general, in Phase 1, the initial introduction of the drug into healthy human volunteers or, in some cases, patients, the drug is tested to assess metabolism, pharmacokinetics, pharmacological actions, side effects associated with increasing doses and, if possible, early evidence of effectiveness. Phase 2 usually involves trials in a limited patient population to determine the effectiveness of the drug for a particular indication, dosage tolerance and optimum dosage, and to identify common adverse effects and safety risks. If a compound demonstrates evidence of effectiveness and an acceptable safety profile in Phase 2 evaluations, Phase 3 trials are undertaken to obtain the additional information about clinical efficacy and safety in a larger number of patients, typically at geographically dispersed clinical trial sites, to permit the FDA to evaluate the overall benefit-risk relationship of the drug and to provide adequate information for the labeling of the drug. In most cases, the FDA requires two adequate and well-controlled Phase 3 clinical trials to demonstrate the efficacy of the drug. The FDA may, however, determine that a drug is effective based on one clinical trial plus confirmatory evidence. Only a small percentage of investigational drugs complete all three phases and obtain marketing approval. In some cases, the FDA may require post-market studies, known as Phase 4 studies, to be conducted as a condition of approval to gather additional information on the drug's effect in various populations and any side effects associated with long-term use. Depending on the risks posed by the drugs, other post-market requirements may be imposed.

After completion of the required clinical testing, an NDA is prepared and submitted to the FDA. FDA approval of the NDA is required before marketing of the product may begin in the United States. The NDA must include the results of all preclinical, clinical, and other testing and a compilation of data relating to the product's pharmacology, chemistry, manufacture, and controls. The cost of preparing and submitting an NDA is substantial. Under federal law, the submission of most NDAs is additionally subject to a substantial application user fee, subject to certain exceptions and waivers, such as for orphan-designated drugs.

The FDA has 60 days from its receipt of an NDA to determine whether the application will be accepted for filing based on the agency's threshold determination that it is sufficiently complete to permit substantive review. Once the submission is accepted for filing, the FDA begins an in-depth review. Under the statute and implementing regulations, the FDA has 180 days (the initial review cycle) from the date of filing to issue either an approval letter or a complete response letter, unless the review period is adjusted by mutual agreement between the FDA and the applicant or as a result of the applicant submitting a major amendment. In practice, the performance goals established pursuant to the Prescription Drug User Fee Act have effectively extended the initial review cycle beyond 180 days. The FDA's current performance goals call for the FDA to complete review of 90% of standard (non-priority) NDAs within 10 months of receipt and within six months for priority NDAs, but two additional months are added to standard and priority NDAs for a new molecular entity, or NME.

The FDA may also refer applications for novel drug products, or drug products that present difficult questions of safety or efficacy, to an advisory committee, which is typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved. The FDA is not bound by the recommendation of an advisory committee, but it generally follows such recommendations. Before approving an NDA, the FDA will typically inspect one or more clinical sites to assure compliance with GCP. Additionally, the FDA will inspect the facility or the facilities at which the drug is manufactured. The FDA will not approve the product unless compliance with current GMP is satisfactory and the NDA contains data that provide substantial evidence that the drug is safe and effective in the indication studied.

After the FDA evaluates the NDA and the manufacturing facilities, it issues either an approval letter or a complete response letter. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing, or information, in order for the FDA to reconsider the application. If, or when, those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the NDA, the FDA will issue an approval letter. The FDA has committed to reviewing 90% of resubmissions within two to six months depending on the type of information included.

An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. As a condition of NDA approval, the FDA may require a risk evaluation and mitigation strategy, or REMS, to help ensure that the benefits of the drug outweigh the potential risks. REMS can include medication guides, communication plans for health care professionals, and elements to assure safe use, or ETASU. ETASU can include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring, and the use of patient registries. The requirement for a REMS can materially affect the potential market and profitability of the drug. Moreover, product approval may require substantial post-approval testing and surveillance to monitor the drug's safety or efficacy. Once granted, product approvals may be withdrawn if compliance with regulatory standards is not maintained or problems are identified following initial marketing.

Disclosure of Clinical Trial Information

Sponsors of clinical trials of certain FDA-regulated products, including prescription drugs, are required to register and disclose certain clinical trial information on a public website maintained by the U.S. National Institutes of Health. Information related to the product, patient population, phase of investigation, study sites and investigator, and other aspects of the clinical trial is made public as part of the registration. Sponsors are also obligated to disclose the results of these trials after completion. Disclosure of the results of these trials can be delayed for up to two years if the sponsor certifies that it is seeking approval of an unapproved product or that it will file an application for approval of a new indication for an approved product within one year. Competitors may use this publicly available information to gain knowledge regarding the design and progress of the development programs.

The Hatch-Waxman Act

Orange Book Listing

In seeking approval for a drug through an NDA, applicants are required to list with the FDA each patent whose claims cover the applicant's product. Upon approval of a drug, each of the patents listed in the application for the drug is then published in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book. Drugs listed in the Orange Book can, in turn, be cited by potential generic competitors in support of approval of an abbreviated new drug application, or ANDA. An ANDA provides for marketing of a drug product that has the same active ingredients in the same strengths and dosage form as the listed drug and has been shown through bioequivalence testing to be bioequivalent to the listed drug. Other than the requirement for bioequivalence testing, ANDA applicants are not required to conduct, or submit results of, pre-clinical or clinical tests to prove the safety or effectiveness of their drug product. Drugs approved in this way are considered to be therapeutically equivalent to the listed drug, are commonly referred to as "generic equivalents" to the listed drug, and can often be substituted by pharmacists under prescriptions written for the original listed drug in accordance with state law.

The ANDA applicant is required to certify to the FDA concerning any patents listed for the approved product in the FDA's Orange Book. Specifically, the applicant must certify that: (i) the required patent information has not been filed; (ii) the listed patent has expired; (iii) the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or (iv) the listed patent is invalid or will not be infringed by the new product. The ANDA applicant may also elect to submit a section viii statement, certifying that its proposed ANDA labeling does not contain (or carves out) any language regarding the patented method-of-use, rather than certify to a listed method-of-use patent.

If the applicant does not challenge the listed patents, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired.

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

The ANDA application also will not be approved until any applicable non-patent exclusivity listed in the Orange Book for the referenced product has expired.

Exclusivity

Upon NDA approval of a new chemical entity, or NCE, which is a drug that contains no active moiety that has been approved by the FDA in any other NDA, that drug receives five years of marketing exclusivity during which time the FDA cannot receive any ANDA or 505(b)(2) application seeking approval of a drug that references a version of the NCE drug. Certain changes to a drug, such as the addition of a new indication to the package insert, are associated with a three-year period of exclusivity during which the FDA cannot approve an ANDA or 505(b)(2) application that includes the change.

An ANDA or 505(b)(2) application may be submitted one year before NCE exclusivity expires if a Paragraph IV certification is filed. If there is no listed patent in the Orange Book, there may not be a Paragraph IV certification and thus no ANDA or 505(b)(2) application may be filed before the expiration of the exclusivity period.

For a botanical drug, the FDA may determine that the active moiety is one or more of the principal components or the complex mixture as a whole. This determination would affect the utility of any five-year exclusivity as well as the ability of any potential generic competitor to demonstrate that it is the same drug as the original botanical drug.

Five-year and three-year exclusivities do not preclude FDA approval of a 505(b)(1) application for a duplicate version of the drug during the period of exclusivity, provided that the 505(b)(1) applicant conducts or obtains a right of reference to all of the preclinical studies and adequate and well controlled clinical trials necessary to demonstrate safety and effectiveness.

Patent Term Extension

After NDA approval, owners of relevant drug patents may apply for up to a five-year patent extension. The allowable patent term extension is calculated as half of the drug's testing phase—the time between IND submission and NDA submission—and all of the review phase—the time between NDA submission and approval up to a maximum of five years. The time can be shortened if the FDA determines that the applicant did not pursue approval with due diligence. The total patent term after the extension may not exceed 14 years.

For patents that might expire during the application phase, the patent owner may request an interim patent extension. An interim patent extension increases the patent term by one year and may be renewed up to four times. For each interim patent extension granted, the post-approval patent extension is reduced by one year. The director of the USPTO must determine that approval of the drug covered by the patent for which a patent extension is being sought is likely. Interim patent extensions are not available for a drug for which an NDA has not been submitted.

Advertising and Promotion

Once an NDA is approved, a product will be subject to certain post-approval requirements. For instance, the FDA closely regulates the post-approval marketing and promotion of drugs.

Drugs may be marketed only for the approved indications and in accordance with the provisions of the approved labeling. Changes to some of the conditions established in an approved application, including changes in indications, labeling, or manufacturing processes or facilities, require submission and FDA approval of a new NDA or NDA supplement before the change can be implemented. An NDA supplement for a new indication typically requires clinical data similar to that in the original application, and the FDA uses the same procedures and actions in reviewing NDA supplements as it does in reviewing NDAs.

Adverse Event Reporting and GMP Compliance

Adverse event reporting and submission of periodic reports is required following FDA approval of an NDA. The FDA also may require post-marketing testing, known as Phase 4 testing, require a REMS special communications regarding the safety of the drug or heightened surveillance to monitor the effects of an approved product, or may place conditions on an approval that could restrict the distribution or use of the product. In addition, quality control, drug manufacture, packaging, and labeling procedures must continue to conform to GMP after approval. Drug manufacturers and certain of their subcontractors are required to register their establishments with the FDA and certain state agencies. Registration with the FDA subjects entities to periodic unannounced inspections by the FDA, during which the agency inspects manufacturing facilities to assess compliance with GMP. Accordingly, manufacturers must continue to expend time, money and effort in the areas of production and quality control to maintain compliance with GMP. Regulatory authorities may withdraw product approvals or request product recalls if a company fails to comply with regulatory standards, if it encounters problems following initial marketing or if previously unrecognized problems are subsequently discovered.

Pediatric Exclusivity and Pediatric Use

The Best Pharmaceuticals for Children Act, or BPCA, provides NDA holders a six-month period of exclusivity attached to any other exclusivity listed with FDA—patent or non-patent—for a drug if certain conditions are met. Conditions for pediatric exclusivity include a determination by the FDA that information relating to the use of a new drug in the pediatric population may produce health benefits in that population; a written request by the FDA for pediatric studies; and agreement by the applicant to perform the requested studies and the submission to the FDA, completion of the studies in accordance with the written request, and the acceptance by the FDA of the reports of the requested studies within the statutory timeframe. Applications under the BPCA are treated as priority applications.

In addition, under the Pediatric Research Equity Act, or PREA, NDAs or supplements to NDAs must contain data to assess the safety and effectiveness of the drug for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the drug is safe and effective, unless the sponsor has received a deferral or waiver from the FDA. Unless otherwise required by regulation, PREA does not apply to any drug for an indication for which orphan designation has been granted. The sponsor or the FDA may request a deferral of pediatric studies for some or all of the pediatric subpopulations. A deferral may be granted for several reasons, including a finding that the drug is ready for approval for use in adults before pediatric studies are complete or that additional safety or effectiveness data need to be collected before the pediatric studies begin. Under PREA, the FDA must send a non-compliance letter requesting a response within 45 days to any sponsor that fails to submit the required assessment, fails to keep a deferral current or fails to submit a request for approval of a pediatric formulation.

Orphan Drugs

Under the Orphan Drug Act, the FDA may grant orphan drug designation to drugs intended to treat a rare disease or condition—generally a disease or condition that affects fewer than 200,000 individuals in the United States (or affects more than 200,000 in the United States and for which there is no reasonable expectation that the cost of developing and making available in the United States a drug for such disease or condition will be recovered from sales of such drug in the United States). Orphan drug designation must be requested before submitting an NDA. After the FDA grants orphan drug designation, the generic identity of the drug and its potential orphan use are disclosed publicly by the FDA. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. The first NDA applicant to receive FDA approval for a particular active ingredient to treat a particular disease with FDA orphan drug designation is entitled to a seven-year exclusive marketing period in the United States for that product, for that indication. During the seven-year exclusivity period, the FDA may not approve any other applications to market the same drug for the same disease, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity. If the FDA designates an orphan drug based on a finding of clinical superiority, the FDA must provide a written notification to the sponsor that states the basis for orphan designation, including "any plausible hypothesis" relied upon by the FDA. The FDA must also publish a summary of its clinical superiority findings upon granting orphan drug exclusivity based on clinical superiority.

Orphan drug exclusivity does not prevent the FDA from approving a different drug for the same disease or condition, or the same drug for a different disease or condition. Among the other benefits of orphan drug designation are tax credits for certain research and a waiver of the NDA application user fee.

Europe/Rest of World Government Regulation

In addition to regulations in the United States, we are and will be subject, either directly or through our distribution partners, to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of our products, if approved.

Whether or not we obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in non-U.S. countries prior to the commencement of clinical trials or marketing of the product in those countries.

In the European Union, medicinal products are subject to extensive pre- and post-marketing regulation by regulatory authorities at both the European Union and national levels. Additional rules also apply at the national level to the manufacture, import, export, storage, distribution and sale of controlled substances. In many E.U. member states, the regulatory authority responsible for medicinal products is also responsible for controlled substances. Responsibility is, however, split in some member states, such as the United Kingdom. Generally, any company manufacturing or distributing a medicinal product containing a controlled substance in the European Union will need to hold a controlled substances license from the competent national authority and will be subject to specific record-keeping and security obligations. Separate import or export certificates are required for each shipment into or out of the member state.

Clinical Trials and Marketing Approval

Certain countries outside of the United States have a process that requires the submission of a clinical trial application much like an IND prior to the commencement of human clinical trials. In Europe, for example, a clinical trial application, or CTA, must be submitted to the competent national health authority and to independent ethics committees in each country in which a company intends to conduct clinical trials. Once the CTA is approved in accordance with a country's requirements and a company has received favorable ethics committee approval, clinical trial development may proceed in that country.

The requirements and process governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country, even though there is already some degree of legal harmonization in the European Union member states resulting from the national implementation of underlying E.U. legislation. In all cases, the clinical trials must be conducted in accordance with the International Conference on Harmonization, or ICH, guidelines on GCP and other applicable regulatory requirements.

To obtain regulatory approval to place a drug on the market in the European Union, we must submit a marketing authorization application. This application is similar to the NDA in the United States, with the exception of, among other things, country-specific document requirements. All application procedures require an application in the common technical document, or CTD, format, which includes the submission of detailed information about the manufacturing and quality of the product, and non-clinical and clinical trial information. Drugs can be authorized in the European Union by using (i) the centralized authorization procedure, (ii) the mutual recognition procedure, (iii) the decentralized procedure or (iv) national authorization procedures.

The European Commission created the centralized procedure for the approval of human drugs to facilitate marketing authorizations that are valid throughout the European Union and, by extension (after national implementing decisions) in Iceland, Liechtenstein and Norway, which, together with the E.U. member states, comprise the European Economic Area, or EEA. Applicants file marketing authorization applications with the EMA, where they are reviewed by a relevant scientific committee, in most cases the Committee for Medicinal Products for Human Use, or CHMP. The EMA forwards CHMP opinions to the European Commission, which uses them as the basis for deciding whether to grant a marketing authorization. This procedure results in a single marketing authorization granted by the European Commission that is valid across the European Union, as well as in Iceland, Liechtenstein and Norway. The centralized procedure is compulsory for human drugs that are: (i) derived from biotechnology processes, such as genetic engineering, (ii) contain a new active substance indicated for the treatment of certain diseases, such as HIV/AIDS, cancer, diabetes, neurodegenerative diseases, autoimmune and other immune dysfunctions and viral diseases, (iii) officially designated "orphan drugs" (drugs used for rare human diseases) and (iv) advanced-therapy medicines, such as gene-therapy, somatic cell-therapy or tissue-engineered medicines. The centralized procedure may at the voluntary request of the applicant also be used for human drugs which do not fall within the above-mentioned categories if the CHMP agrees that (a) the human drug contains a new active substance not yet approved on November 20, 2005; (b) it constitutes a significant therapeutic, scientific or technical innovation or (c) authorization under the centralized procedure is in the interests of patients at the E.U. level.

Under the centralized procedure in the European Union, the maximum timeframe for the evaluation of a marketing authorization application by the EMA is 210 days (excluding clock stops, when additional written or oral information is to be provided by the applicant in response to questions asked by the CHMP), with adoption of the actual marketing authorization by the European Commission thereafter. Accelerated evaluation might be granted by the CHMP in exceptional cases, when a medicinal product is expected to be of a major public health interest from the point of view of therapeutic innovation, defined by three cumulative criteria: the seriousness of the disease to be treated, the absence of an appropriate alternative therapeutic approach, and anticipation of exceptional high therapeutic benefit. In this circumstance, the EMA ensures that the evaluation for the opinion of the CHMP is completed within 150 days and the opinion issued thereafter.

For those medicinal products for which the centralized procedure is not available, the applicant must submit marketing authorization applications to the national medicines regulators through one of three procedures: (i) the mutual recognition procedure (which must be used if the product has already been authorized in at least one other E.U. member state, and in which the E.U. member states are required to grant an authorization recognizing the existing authorization in the other E.U. member state, unless they identify a serious risk to public health), (ii) the decentralized procedure (in which applications are submitted simultaneously in two or more E.U. member states) or (iii) national authorization procedures (which results in a marketing authorization in a single E.U. member state).

Mutual Recognition Procedure

The mutual recognition procedure, or MRP, for the approval of human drugs is an alternative approach to facilitate individual national marketing authorizations within the European Union. Basically, the MRP may be applied for all human drugs for which the centralized procedure is not obligatory. The MRP is applicable to the majority of conventional medicinal products and must be used if the product has already been authorized in one or more member states.

The characteristic of the MRP is that the procedure builds on an already–existing marketing authorization in a member state of the European Union that is used as a reference in order to obtain marketing authorizations in other E.U. member states. In the MRP, a marketing authorization for a drug already exists in one or more member states of the European Union and subsequently marketing authorization applications are made in other E.U. member states by referring to the initial marketing authorization. The member state in which the marketing authorization was first granted will then act as the reference member state. The member states where the marketing authorization is subsequently applied for act as concerned member states. The concerned member states are required to grant an authorization recognizing the existing authorization in the reference member state, unless they identify a serious risk to public health.

The MRP is based on the principle of the mutual recognition by E.U. member states of their respective national marketing authorizations. Based on a marketing authorization in the reference member state, the applicant may apply for marketing authorizations in other member states. In such case, the reference member state shall update its existing assessment report about the drug in 90 days. After the assessment is completed, copies of the report are sent to all member states, together with the approved summary of product characteristics, labeling and package leaflet. The concerned member states then have 90 days to recognize the decision of the reference member state and the summary of product characteristics, labeling and package leaflet. National marketing authorizations shall be granted within 30 days after acknowledgement of the agreement.

If any E.U. member state refuses to recognize the marketing authorization by the reference member state, on the grounds of potential serious risk to public health, the issue will be referred to a coordination group. Within a timeframe of 60 days, member states shall, within the coordination group, make all efforts to reach a consensus. If this fails, the procedure is submitted to an EMA scientific committee for arbitration. The opinion of this EMA Committee is then forwarded to the European Commission for the start of the decision making process. As in the centralized procedure, this process entails consulting various European Commission Directorates General and the Standing Committee on Human Medicinal Products.

Data Exclusivity

In the European Union, marketing authorization applications for generic medicinal products do not need to include the results of pre-clinical and clinical trials, but instead can refer to the data included in the marketing authorization of a reference product for which regulatory data exclusivity has expired. If a marketing authorization is granted for a medicinal product containing a new active substance, that product benefits from eight years of data exclusivity, during which generic marketing authorization applications referring to the data of that product may not be accepted by the regulatory authorities, and a further two years of market exclusivity, during which such generic products may not be placed on the market. The two-year period may be extended to three years if during the first eight years a new therapeutic indication with significant clinical benefit over existing therapies is approved.

Orphan Medicinal Products

The EMA's Committee for Orphan Medicinal Products, or COMP, may recommend orphan medicinal product designation to promote the development of products that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than five in 10,000 persons in the European Union. Additionally, this designation is granted for products intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the product in the European Union would be sufficient to justify the necessary investment in developing the medicinal product. The COMP may only recommend orphan medicinal product designation when the product in question offers a significant clinical benefit over existing approved products for the relevant

indication. Following a positive opinion by the COMP, the European Commission adopts a decision granting orphan status. The COMP will reassess orphan status in parallel with EMA review of a marketing authorization application and orphan status may be withdrawn at that stage if it no longer fulfills the orphan criteria (for instance because in the meantime a new product was approved for the indication and no convincing data are available to demonstrate a significant benefit over that product). Orphan medicinal product designation entitles a party to financial incentives such as a reduction of fees or fee waivers and 10 years of market exclusivity is granted following marketing authorization. During this period, the competent authorities may not accept or approve any similar medicinal product, unless it offers a significant clinical benefit. This period may be reduced to six years if the orphan medicinal product designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity.

Pediatric Development

In the European Union, companies developing a new medicinal product must agree to a Paediatric Investigation Plan, or PIP, with the EMA and must conduct pediatric clinical trials in accordance with that PIP unless a waiver applies, for example, because the relevant disease or condition occurs only in adults. The marketing authorization application for the product must include the results of pediatric clinical trials conducted in accordance with the PIP, unless a waiver applies, or a deferral has been granted, in which case the pediatric clinical trials must be completed at a later date. Products that are granted a marketing authorization on the basis of the pediatric clinical trials conducted in accordance with the PIP are eligible for a six-month extension of the protection under a supplementary protection certificate (if the product covered by it qualifies for one at the time of approval). This pediatric reward is subject to specific conditions and is not automatically available when data in compliance with the PIP are developed and submitted.

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

Reimbursement

Sales of pharmaceutical products in the United States will depend, in part, on the extent to which the costs of the products will be covered by third-party payers, such as government health programs, and commercial insurance and managed health care organizations. These third-party payers are increasingly challenging the prices charged for medical products and services. Additionally, the containment of health care costs has become a priority of federal and state governments, and the prices of drugs have been a focus in this effort. The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs, including price controls, utilization management and requirements for substitution of generic products. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit our net revenue and results. If these third-party payers do not consider our products to be cost-effective compared to other available therapies, they may not cover our products after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow us to sell our products on a profitable basis.

The Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or the MMA, imposed requirements for the distribution and pricing of prescription drugs for Medicare beneficiaries and included a major expansion of the prescription drug benefit under Medicare Part D. Under Part D, Medicare beneficiaries may enroll in prescription drug plans offered by private entities that provide coverage of outpatient prescription drugs. Part D is available through both stand-alone prescription drug benefit plans and prescription drug coverage as a supplement to Medicare Advantage plans. Unlike Medicare Parts A and B, Part D coverage is not standardized. Part D prescription drug plan sponsors are not required to pay for all covered Part D drugs, and each drug plan can develop its own drug formulary that identifies which drugs it will cover and at what tier or level. However, Part D prescription drug formularies must include drugs within each therapeutic category and class of covered Part D drugs, though not necessarily all the drugs in each category or class. Any formulary used by a Part D prescription drug plan must be developed and reviewed by a pharmacy and therapeutic committee.

Government payment for some of the costs of prescription drugs may increase demand for products for which we receive marketing approval. However, any negotiated prices for our products covered by a Part D prescription drug plan will likely be lower than the prices we might otherwise obtain. Moreover, while the MMA applies only to drug benefits for Medicare beneficiaries, private payers often follow Medicare coverage policy and payment limitations in setting their own payment rates. Any reduction in payment that results from the MMA may result in a similar reduction in payments from non-governmental payers.

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act of 2010, collectively, the ACA, was enacted with the goal of expanding coverage for the uninsured while at the same time containing overall health care costs. With regard to pharmaceutical products, among other things, the ACA expanded and increased industry rebates for drugs covered under Medicaid programs and made changes to the coverage requirements under the Medicare D program. We still cannot fully predict the impact of the ACA on pharmaceutical companies as many of the ACA reforms require the promulgation of detailed regulations implementing the statutory provisions which has not yet been completed, and the Centers for Medicare & Medicaid Services has publicly announced that it is analyzing the ACA regulations and policies that have been issued to determine if changes should be made. In addition, although the United States Supreme Court has upheld the constitutionality of most of the ACA, some states have stated their intentions to not implement certain sections of the ACA and some members of Congress and President Trump are still working to repeal the ACA. These challenges add to the uncertainty of the changes enacted as part of ACA.

In addition, in some foreign countries, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country. For example, some E.U. jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical trials that compare the cost-effectiveness of a particular product candidate to currently available therapies. Other member states allow companies to fix their own prices for medicines but monitor and control company profits. Such differences in national pricing regimes may create price differentials between E.U. member states. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products. Historically, products launched in the European Union do not follow price structures of the United States. In the European Union, the downward pressure on healthcare costs in general, particularly prescription medicines, has become intense. As a result, barriers to entry of new products are becoming increasingly high and patients are unlikely to use a drug product that is not reimbursed by their government.

New Legislation and Regulations

From time to time, legislation is drafted, introduced and passed in Congress that could significantly change the statutory provisions governing the testing, approval, manufacturing and marketing of products regulated by the FDA and relevant regulatory authorities outside the United States. In addition to new legislation, regulations and policies are often revised or interpreted by regulatory authorities in ways that may significantly affect our business and our product candidates. It is impossible to predict whether further legislative changes will be enacted or whether regulations, guidance, policies or interpretations will be changed or what the effect of such changes, if any, may be.

Pharmaceutical Coverage, Pricing and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any drug products for which we obtain regulatory approval. In the United States, sales of any products for which we receive regulatory approval for commercial sale will depend in part on the availability of coverage and reimbursement from third-party payors. Third-party payors include government authorities, managed care providers, private health insurers and other organizations. The process for determining whether a payor will provide coverage for a drug product may be separate from the process for setting the reimbursement rate that the payor will pay for the drug product. Third-party payors may limit coverage to specific drug products on an approved list, or formulary, which might not include all of the FDA-approved drugs for a particular indication. Moreover, a payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

Third-party payors are increasingly challenging the price and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. In order to obtain coverage and reimbursement for any product that might be approved for sale, we may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain regulatory approvals. Our product candidates may not be considered medically necessary or cost-effective. If third-party payors do not consider a product to be cost-effective compared to other available therapies, they may not cover the product after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow a company to sell its products at a profit.

Other Healthcare Laws and Compliance Requirements

If we obtain regulatory approval of our products, we may be subject to various federal and state laws targeting fraud and abuse in the healthcare industry. These laws may impact, among other things, our proposed sales, marketing and education programs. In addition, we may be subject to patient privacy regulation by both the federal government and the states in which we conducts our business. The laws that may affect our ability to operate include:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, to induce, or in return for, the purchase or recommendation of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs;
- federal civil and criminal false claims laws and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid, or other third-party payers that are false or fraudulent;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created new federal criminal statutes that prohibit executing a scheme to defraud any healthcare benefit program and making false statements relating to healthcare matters;
- the federal transparency laws, including the federal Physician Payments Sunshine Act, that require drug manufacturers to disclose payments and other transfers of value provided to physicians and teaching hospitals;
- HIPAA, as amended by the Health Information Technology and Clinical Health Act, or HITECH, and its implementing regulations, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information; and
- state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payer, including commercial insurers, state laws governing the disclosure of payments to health care professionals and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

Employees

As of March 15, 2019, we employed 39 full-time employees, of whom 14 hold Ph.D. or M.D. degrees. Of these employees, 27 were engaged in research and development and 12 were engaged in general and administrative functions. All of our employees are located in the United States and Vienna, Austria. We have no collective bargaining agreements with our employees and have not experienced any work stoppages. We consider our relationship with our employees to be good.

Facilities

We lease approximately 12,577 square feet of office space at 955 Massachusetts Avenue, 4th Floor, Cambridge, Massachusetts, which serves as our corporate headquarters. The lease expires on July 31, 2022, and we have the option to extend the term one time for an additional five-year period. The base monthly payment on the lease is $67,077 as of December 31, 2018, subject to specified annual increases of approximately 1.5% during the

term of the lease and not including operating expenses, certain utilities, taxes and insurance for which we are responsible. In addition, we currently lease approximately 5,711 square feet of office space in Waltham, Massachusetts under a lease that currently expires in December 2023 and approximately 410 square meters of office and laboratory space in Vienna, Austria under a sublease that currently expires in February 2021. We believe that our existing facilities are adequate to meet our current needs and that suitable alternative spaces will be available in the future on commercially reasonable terms. Following the Merger, we are consolidating our U.S. operations into our corporate headquarters in Cambridge, Massachusetts. As a result, we intend to seek to sublease our facility in Waltham, Massachusetts. There can be no assurances, however, that we will be able to sublease all or any portion of this facility on acceptable terms, or at all.

Legal Proceedings

We are not currently a party to any material legal proceedings.

Special Note Regarding Forward-Looking Statements

This business section contains "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, that relate to future events or to our future operating or financial performance. Any forward-looking statement involves known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to differ materially from any future results, levels of activity, performance or achievements expressed or implied by such forward-looking statement. Forward-looking statements include statements, other than statements of historical fact, about, among other things:

- the progress, scope, cost, duration or results of our development activities, nonclinical studies and clinical trials of mavorixafor (X4P-001), X4P-002 and X4P-003 or any of our other product candidates or programs, such as the target indication(s) for development, the size, design, population, conduct, cost, objective or endpoints of any clinical trial, or the timing for initiation or completion of or availability of results from any clinical trial (including our planned trials for mavorixafor in Warts, Hypogammaglobulinemia, Infections, and Myelokathexis syndrome, severe congenital neutropenia and Waldenström macroglobulinemia), for submission or approval of any regulatory filing or for meeting with regulatory authorities;
- our plans with respect to the Arsanis preclinical and clinical programs;
- 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, any product candidates that we may develop, and any related restrictions, limitations, or warnings in the label of any approved product candidates;
- our future operations, financial position, revenues, costs, expenses, uses of cash, capital requirements or our need for additional financing; and
- our strategies, prospects, plans, expectations or objectives.

Words such as, but not limited to, "believe," "expect," "anticipate," "estimate," "forecast," "intend," "may," "plan," "potential," "predict," "project," "targets," "likely," "will," "would," "could," "should," "continue," "scheduled" and similar expressions or phrases, or the negative of those expressions or phrases, are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Although we believe that we have a reasonable basis for each forward-looking statement contained in this report, we caution you that these statements are based on our projections of the future that are subject to known and unknown risks and uncertainties and other important factors that may cause our actual results, level of activity, performance or achievements expressed or implied by any forward-looking statement to differ. These risks, uncertainties and other factors are described in greater detail under the caption "Risk Factors" in the filings that we make with the Securities and Exchange Commission. Also, these forward-looking statements represent our estimates and assumptions only as of the date of the document containing the applicable statement. As a result of the risks and uncertainties, the results or events indicated by the forward-looking statements may not occur. We caution you not to place undue reliance on any forward-looking statement.

You should read this report and the documents we have filed with the SEC that are incorporated by reference completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of the forward-looking statements in the foregoing documents by these cautionary statements. Unless required by law, we undertake no obligation to update or revise any forward-looking statements to reflect new information or future events or developments. Thus, you should not assume that our silence over time means that actual events are bearing out as expressed or implied in such forward-looking statements.

Exhibit 99.2

RISK FACTORS OF X4 PHARMACEUTICALS, INC.

An investment in shares of our common stock involves a high degree of risk. You should carefully consider the following information about these risks, together with the other information appearing in our Annual Report on Form 10-K for the year ended December 31, 2018 and our subsequent filings with the Securities and Exchange Commission, or SEC, including the audited consolidated financial statements and related notes and the pro forma financial statements attached as exhibits to Amendment No. 1 to the Current Report on Form 8-K/A filed with the SEC on April 3, 2019, 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. In these circumstances, the market price of our common stock could decline, and you may lose all or part of your investment.

On March 13, 2019, X4 Pharmaceuticals, Inc., formerly Arsanis, Inc., or the Company, completed its business combination with X4 Therapeutics, Inc., formerly X4 Pharmaceuticals, Inc., or X4, in accordance with the terms of the Agreement and Plan of Merger, dated as of November 26, 2018, as amended on December 20, 2018 and March 8, 2019, or the Merger Agreement, by and among the Company, X4 and Artemis AC Corp., a Delaware corporation and a wholly owned subsidiary of the Company, or Merger Sub, pursuant to which, among other matters, Merger Sub merged with and into X4, with X4 continuing as a wholly owned subsidiary of the Company and the surviving corporation of the merger, which we refer to as the Merger. Following the closing of the Merger, on March 13, 2019, the Company effected a 6-for-1 reverse stock split of its common stock, which we refer to as the Reverse Stock Split, and changed its name to X4 Pharmaceuticals, Inc. Following the completion of the Merger, the business conducted by the Company became primarily the business conducted by X4, which is a clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of novel therapeutics for the treatment of rare diseases. As used herein, the words "the Company," "we," "us," and "our" refer to X4 Pharmaceuticals, Inc. (formerly Arsanis, Inc.) and its direct and indirect subsidiaries, as applicable. In addition, the word "Arsanis" refers to the Company prior to the completion of the Merger. Unless noted otherwise, all references herein to share and per share amounts reflect the Reverse Stock Split.

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 and may never generate profits from operations or maintain profitability.

Since inception, we have incurred significant operating losses. Without regard to the historical operating results of our predecessor, Arsanis, our net losses were $22.0 million and $33.3 million for the years ended December 31, 2017 and 2018, respectively, and we had an accumulated deficit of $79.2 million as of December 31, 2018. To date, we have financed our operations primarily through issuances of shares of common stock, preferred stock or convertible notes and through borrowing under our prior loan and security agreement with Silicon Valley Bank and under our existing loan and security agreement with Hercules Capital, Inc. We have not generated any revenue from product sales to date. We have devoted substantially all of our efforts to research and development, including clinical trials. We have not completed the development of any drugs. 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, as well as to support our transition to a public reporting company. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenues. 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 initiation and completion of the Phase 3 trial of mavorixafor (X4P-001) for the treatment of WHIM syndrome, our Phase 1/2 clinical trial of mavorixafor for the treatment of severe congenital neutropenia, or SCN, our Phase 1/2 clinical trial of mavorixafor for the treatment of Waldenström macroglobulinemia, or WM, and our Phase 1/2 clinical trial for the treatment of clear cell renal cell carcinoma, or ccRCC;
- 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.

Our limited operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

Without regard to the historical operations of Arsanis, our operations to date have been limited to organizing the company, entering into licensing arrangements for mavorixafor, hiring a team of experienced personnel, raising capital, and undertaking nonclinical studies and clinical trials and regulatory activities for our development programs, primarily mavorixafor. We have not yet demonstrated our ability to successfully complete development of any product candidate, including large-scale, pivotal clinical trials required for regulatory approval of our product candidates, obtain marketing approvals, manufacture a commercial scale product, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization.

Typically, it takes many years to develop one new product from the time it is discovered to when it is commercially available, if ever. In addition, the CXCR4 receptor that we are pursuing with respect to our product candidates is a relatively novel target with a limited research and development history. Consequently, any early predictions made about our future success or viability may not be as accurate as they could be if we had a longer operating history.

As an early-stage company, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors that may alter or delay our plans. Assuming 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 substantially 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 in the United States. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations.

We cannot be certain that additional funding will be available on acceptable terms, or at all. If we are unable to raise additional capital when needed or in sufficient amounts or on terms acceptable to it, 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 the Phase 3 trial of mavorixafor for the treatment of WHIM syndrome, our Phase 1/2 clinical trial of mavorixafor for the treatment of SCN, our Phase 1/2 clinical trial of mavorixafor for the treatment of WM, and our Phase 1/2 for the treatment of ccRCC;
- 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 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;
- 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 in the United States, 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.

Conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of products that will not be commercially available for sale by us for at least the next few years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. In addition, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans. Additional financing may not be available to us on acceptable terms, or at all. The unavailability of additional financing on acceptable terms, or at all, would have an adverse effect on your investment.

X4's independent registered public accounting firm has included an explanatory paragraph relating to X4's ability to continue as a going concern in its report on X4's audited financial statements for the year ended December 31, 2018.

The report from X4's independent registered public accounting firm for the year ended December 31, 2018 includes an explanatory paragraph stating that X4's recurring losses from operations and required additional funding to finance X4's operations raise substantial doubt about X4's ability to continue as a going concern. If X4 is unable to obtain sufficient funding, its business, prospects, financial condition and results of operations will be materially and adversely affected and X4 and the Company may be unable to continue as a going concern. If X4 is unable to continue as a going concern, X4 may have to liquidate its assets and may receive less than the value at which those assets are carried on its audited financial statements, and it is likely that investors will lose all or a part of their investment. Future reports from our independent registered public accounting firm may also contain statements expressing substantial doubt about our ability to continue as a going concern. If we seek additional financing to fund our business activities in the future and there remains substantial doubt about our ability to continue as a going concern, investors or other financing sources may be unwilling to provide additional funding to us on commercially reasonable terms or at all.

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 a combination of equity offerings, debt financings, and licensing, collaboration or similar arrangements. 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 since inception and may never become profitable.

To date, we have not generated any revenues. 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 other 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 and Commercialization 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 WM and 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 drug product for sale and may never be able to develop marketable drug products. We plan to initiate 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. Mavorixafor for the treatment of SCN and for the treatment of WM has not yet entered clinical development and mavorixafor for the treatment of ccRCC has not yet entered Phase 3 clinical development. We also intend to pursue a collaboration or other third party arrangement for future development and potential commercialization of mavorixafor in ccRCC, and we do not plan to develop mavorixafor for the treatment of ccRCC 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 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 the Phase 3 pivotal clinical trial of mavorixafor in patients with WHIM syndrome and potentially additional clinical trials and/or nonclinical studies. 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, including disagreement with our plan to conduct a single pivotal Phase 3 trial of mavorixafor in patients with WHIM syndrome;
- 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.

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 (www.clinicaltrials.gov or EU's clinical trial database EudraCT). Competitors may use this publicly available information to gain knowledge regarding the progress of development programs.

Our lead product candidate, mavorixafor, is only part way through the clinical trials we anticipate needing to complete before we may be able to submit an NDA to the FDA. Clinical drug development involves a lengthy and expensive process with an uncertain outcome, and results of early studies and trials may not be predictive of later trial results.

Preclinical and other nonclinical testing and clinical trials are long, expensive and unpredictable processes that are difficult to design and implement, are subject to delays and are uncertain as to outcome. It may take several years to complete the nonclinical testing and clinical development necessary to obtain approval and commercialize a drug, and failure can occur at any stage of testing. Early and interim results of clinical trials do not necessarily predict final results. In particular, the small number of subjects and patients in our early clinical trials may make the results of these clinical trials less predictive of the outcome of later larger clinical trials. The design of a clinical trial may be able to determine whether its results will support approval of a product, and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced or completed. There is no assurance that we will be able to design and complete a clinical trial to support marketing approval. Moreover, nonclinical and clinical data are often susceptible to multiple interpretations and analyses. A number of companies in the pharmaceutical and biotechnology industries have experienced significant setbacks in advanced clinical trials, even after promising results in earlier trials.

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 1/2 clinical trial of mavorixafor for the treatment of SCN, our Phase 1/2 clinical trial of mavorixafor for the treatment of WM, and our Phase 1/2 for the treatment of ccRCC. 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, such as:

- 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. The timing of our clinical trials depends in part on the speed at which we can recruit patients to participate in testing mavorixafor and any other current or future product candidates that we may develop as well as completion of required follow-up periods. If we cannot identify patients to participate in our clinical trials or if patients are unwilling to participate in our clinical trials for any reason, including if patients choose to enroll in competitive clinical trials for similar patient populations, the timeline for recruiting patients, conducting studies and obtaining regulatory approval of mavorixafor and any other current or future product candidates that we may develop may be delayed. These delays could result in increased costs, delays in advancing our current or future product candidates, 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, an orphan disease 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:

- 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;
- patient referral practices of physicians; and
- our ability to monitor patients adequately during and after treatment.

We have made certain assumptions about the rate at which we can enroll patients in our clinical trials. To the extent that we do not meet this enrollment target, our projected timeline for development of our product candidates may be slowed. We rely on CROs and clinical trial sites to ensure the proper and timely conduct of our clinical trials and while we will have agreements governing their activities, we have limited control over their actual performance.

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 is smaller than we anticipate, our potential future revenue from mavorixafor for the treatment of WHIM syndrome 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 WM. WHIM syndrome, SCN and WM are each rare diseases, with a limited patient population.

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 preliminary independent market research study conducted by a third-party research firm study that we sponsored, we estimate there are more than 1,000 genetically confirmed WHIM patients in the United States. 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 and any other primary immunodeficiency that we may target. Our projections of the number of people who have WHIM syndrome, or its other potential primary immunodeficiencies, 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 WHIM syndrome. The effort to identify patients with WHIM syndrome or our other potential target indications 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 WHIM syndrome 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. Further, even if we obtain significant market share for WHIM syndrome, we may never achieve profitability because the potential target patient population in WHIM syndrome is small.

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:

- 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. Significant preclinical study or clinical trial delays 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 mavorixafor for the treatment of WHIM syndrome, for the treatment of SCN, for the treatment of WM, and for the treatment of ccRCC. 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.

Risks Related to the Marketing and Commercialization of Our Product Candidates

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;
- the availability of adequate reimbursement and pricing 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.

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, 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 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 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 manufacturers of drugs, devices, biologics and medical supplies that are reimbursable under Medicare, Medicaid, or the Children's Health Insurance Program to report to CMS information related to payments and other transfers of value to physicians and teaching hospitals and the ownership and investment interests of physicians and their immediate family members in such manufacturers;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, which also imposes obligations on certain covered entity healthcare providers, health plans, and healthcare clearinghouses as well as their business associates that perform certain services involving the use or disclosure of 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 or marketing expenditures; 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, 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 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 agree to offer 50% 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 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.

The current administration supports a repeal of the ACA and an Executive Order has been signed mandating that federal agencies try to waive or delay requirements of the ACA that impose economic or regulatory burdens on states, families, the health-care industry and others. The Executive Order also declares that the administration will seek the "prompt repeal" of the law and that the government should prepare to "afford the States more flexibility and control to create a more free and open healthcare market." At this time, the immediate impact of the Executive Order is not clear. 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.

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. 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 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.

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 persuade adequate numbers of physicians to prescribe 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. We are also developing mavorixafor for the treatment of SCN, for the treatment of WM and for the treatment of ccRCC. 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 WM, the Dana Farber Cancer Institute has initiated a trial to study the BMS CXCR4 antibody (IV infusion) in the treatment of WM patients with CXCR4 mutations. In WM, 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. Our principal competitors in ccRCC include Pfizer, Novartis, BMS, and Merck. In glioblastoma, our principal competitors include Genentech/Roche and BMS.

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 intend to market mavorixafor and our other product candidates outside of the United States, and if we do, we will be subject to the risks of doing business outside of the United States.

Because we intend to market mavorixafor and other product candidates, if approved, outside of the United States, our business is subject to risks associated with doing business outside of the United States. Accordingly, our business and financial results in the future could be adversely affected due to a variety of factors, including:

- failure to develop an international sales, marketing and distribution system for our products;
- changes in a specific country's or region's political and cultural climate or economic condition;
- unexpected changes in foreign laws and regulatory requirements;
- difficulty of effective enforcement of contractual provisions in local jurisdictions;
- inadequate intellectual property protection in foreign countries;
- inadequate data protection against unfair commercial use;
- trade-protection measures, import or export licensing requirements such as Export Administration Regulations promulgated by the United States Department of Commerce and fines, penalties or suspension or revocation of export privileges;
- the effects of applicable foreign tax structures and potentially adverse tax consequences; and
- significant adverse changes in foreign currency exchange rates.

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 Our Dependence on Third Parties

We have no experience manufacturing our product candidates on a large clinical or commercial scale and have no manufacturing facility. We are currently dependent on a single third party manufacturer for the manufacture of mavorixafor for the active pharmaceutical ingredient, or API, and a single manufacturer of mavorixafor finished drug product capsules, and 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 for the active pharmaceutical ingredient, or API, and a single manufacturer of mavorixafor finished drug product capsules. 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 scale of production or we will need to find additional or alternative manufacturers. We have not yet identified alternate suppliers in the event the current manufacturer we utilize is unable to scale production, 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.

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 for the API 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.

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

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

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

We intend to pursue a strategic collaboration for future development and potential commercialization of mavorixafor in ccRCC. In addition, we are in discussions with respect to a strategic collaboration for the development and potential commercialization of mavorixafor in WHIM syndrome and oncology in Greater China. Although we currently have no definitive agreement with respect to such strategic collaboration, such strategic collaboration could materially impact our business, financial condition and results of operations. No assurance can be given, however, that we will enter into any such strategic collaboration or, if entered into, any such 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 collaborate with a 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.

To the extent we enter into any collaborations, 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 may selectively seek 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, 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, came into effect on September 16, 2012. Substantive changes to patent law associated with the America Invents Act may affect our ability to obtain patents, and, if obtained, to enforce or defend them in litigation or post-grant proceedings, 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 decisions described above, rulings in other cases or changes in guidance or procedures issued by the USPTO. We cannot fully predict what impact the Supreme Court's decisions in Prometheus and Myriad 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. 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 Regulatory Approval of Our Product Candidates and Other Legal and Compliance Matters

If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we will not be able to commercialize, or will be delayed in commercializing, our product candidates, and our ability to generate revenue will be impaired.

Our product candidates and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory agencies in the United States and by comparable authorities in other countries. Failure to obtain marketing approval for a product candidate will prevent us from commercializing the product candidate. We have not received approval to market any of our product candidates from regulatory authorities in any jurisdiction. Securing regulatory approval requires the submission of extensive preclinical and clinical data and supporting information to the various regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. Securing regulatory approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authority. Our product candidates may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude us from obtaining marketing approval or prevent or limit commercial use.

The process of obtaining marketing approvals, both in the United States and elsewhere, is expensive, may take many years and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. We cannot assure you that we will ever obtain any marketing approvals in any jurisdiction. Changes in marketing approval requirements during the development period, changes in or the enactment of additional statutes or regulations or changes in regulatory review for each submitted product application may cause delays in the approval or rejection of an application. The FDA and comparable authorities in other countries have substantial discretion in the review and approval process and may refuse to accept any

application or may decide that our data is insufficient for approval and require additional nonclinical or other studies, and clinical trials. In addition, varying interpretations of the data obtained from preclinical testing and clinical trials could delay, limit or prevent marketing approval of a product candidate. Additionally, any marketing approval that we ultimately may obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable.

Failure to obtain regulatory approval in international jurisdictions would prevent our product candidates from being marketed abroad.

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.

It is possible that we may not be able to obtain or maintain orphan drug designation or exclusivity for our drug 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 we submitted our orphan drug designation request to the EMA for mavorixafor for the treatment of WHIM syndrome in March 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 U.S. 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.

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.

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

We currently have a limited number of employees and our future success depends on our ability to retain our executive officers and to attract, retain and motivate qualified 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 current management, especially our co-founder and President and Chief Executive Officer, Paula Ragan, Ph.D., who is the driving force behind the idea and successful implementation of our development strategy. 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. We intend to increase our technical and management staff as needs arise and supporting resources become available, but the loss of one or more of our executive officers, including their death, could be detrimental to us if we cannot recruit suitable replacements in a timely manner. The competition for qualified personnel in the pharmaceutical field is intense and as a result, we may be unable to continue to attract and retain qualified personnel necessary for the development of our business or to recruit suitable replacement personnel.

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

As of March 15, 2019, we had 31 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. 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. 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 and other natural or manmade 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.

In general, under Section 382 of the Internal Revenue Code of 1986, as amended, or the Code, a corporation that undergoes an "ownership change" is subject to limitations on its ability to utilize its carryforwards to offset future taxable income. Our existing net operating loss carryforwards, or NOLs, may be subject to limitations arising from previous ownership changes, including in connection with the Merger, and if we undergo a subsequent ownership change, our ability to utilize NOLs could be further limited by Section 382 of the Code. Future changes in our stock ownership, some of which are outside of our control, could result in an ownership change under Section 382 of the Code. There is also a risk that due to regulatory changes, such as suspensions on the use of NOLs, or other unforeseen reasons, our existing and any future NOLs could expire or otherwise be unavailable to offset future income tax liabilities.

We have not conducted a study to assess whether an ownership change has occurred or whether there have been multiple ownership changes since inception due to the significant complexity and cost associated with such a study.

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

In October 2018, we entered into a loan and security agreement with Hercules Capital, Inc., 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 maintaining a minimum liquidity amount of the lesser of (i) 125% of outstanding borrowings under the Hercules Loan Agreement and (ii) 100% of the Company's cash and cash equivalents in an account in which Hercules has a first priority security interest. 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.

We will incur costs and demands upon management as a result of complying with the laws and regulations affecting public companies.

We will incur significant legal, accounting and other expenses that we did not incur as a private company, including costs associated with public company reporting requirements. We will also incur costs associated with corporate governance requirements, including requirements under the Sarbanes-Oxley Act, as well as rules implemented by the SEC and Nasdaq. These rules and regulations are expected to increase our legal and financial compliance costs and to make some activities more time consuming and costly. Our executive officers and other personnel will need to devote substantial time regarding operations as a public company and compliance with applicable laws and regulations. These rules and regulations may also make it difficult and expensive for us to obtain and maintain directors' and officers' liability insurance. We have not purchased any key man insurance policies with respect to our executive officers. As a result, it may be more difficult for us to attract and retain qualified individuals to serve on our board of directors or as our executive officers, which may adversely affect investor confidence in us and could cause our business or stock price to suffer.

The tax reform enacted in 2017 could adversely affect our business and financial condition.

On December 22, 2017, President Trump signed into law the "Tax Cuts and Jobs Act," or the TCJA, that significantly reforms the Code. The TCJA, among other things, contains significant changes to corporate taxation, including reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, limitation on the deductibility of interest expense to 30% of adjusted earnings (except for certain small businesses), limitation of the deduction for net operating losses to 80% of current year taxable income and elimination of net operating loss carrybacks, one time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, reduction of U.S. tax on foreign earnings (subject to certain important exceptions), immediate deductions for certain new investments instead of deductions for depreciation expense over time, and modifying or repealing many business deductions and credits. We continue to examine the impact this tax reform legislation may have on our business. The overall impact of the TCJA is uncertain and our business and financial condition could be adversely affected. The impact of this tax reform on holders of our common stock also uncertain and could be adverse. You are urged to consult with your legal and tax advisors with respect to such legislation 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 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; and
- period-to-period fluctuations in our financial results.

Moreover, the stock markets in general have experienced substantial volatility that has often been unrelated to the operating performance of individual companies. These broad market fluctuations may also adversely affect the trading price of our common stock.

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. 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.

We expect to be heavily reliant on our ability to access funding through capital market transactions. Due to our small public float, low market capitalization, limited operating history and lack of revenue, it may be difficult and expensive for us to raise additional funds.

We anticipate that we will be heavily reliant on our ability to raise funds through the issuance of shares of our common stock or securities linked to our common stock. Our ability to raise these funds may be dependent on a number of factors, including the low trading volume and volatile trading price of our shares of common stock and other risk factors described herein. The stocks of small cap companies in the biotechnology sector like us tend to be highly volatile. We expect that the price of our common stock will be highly volatile for the next several years. Even if we expand our portfolio of products and product candidates, we may never successfully commercialize or monetize our current product candidate or any future product candidate that we may seek to develop.

As a result, we may be unable to access funding through sales of our common stock or other equity-linked securities. Even if we were able to access funding, the cost of capital may be substantial due to our low market cap and our small public float. The terms of any funding we are able to obtain may not be favorable to us and may be highly dilutive to our stockholders. We may be unable to access capital due to unfavorable market conditions or other market factors outside of our control. There can be no assurance that we will be able to raise additional capital when needed. The failure to obtain additional capital when needed would have a material adverse effect on our business.

The changes to our board of directors resulting from the Merger may affect our business strategy and operations.

The composition of our board of directors changed in connection with the Merger. On March 13, 2019, immediately prior to and effective upon the closing of the Merger, Michael P. Gray, William Clark, M.B.A, Tillman U. Gerngross, Ph.D., Carl Gordon, Ph.D., C.F.A., Terrance McGuire, Claudio Nessi, Ph.D., M.B.A., Michael Ross, Ph.D. and Amy Schulman, J.D. resigned from our board of directors and committees of the board of directors on which they respectively served, In accordance with the Merger Agreement, at the closing of the Merger on March 13, 2019, the board of directors was reconstituted, with our board of directors electing Paula Ragan, Ph.D., X4's President and Chief Executive Officer, Michael S. Wyzga, the chairman of X4's board of directors, and Isaac Blech and Gary J. Bridger, directors who served on X4's board of directors, to our board of directors to serve on the board with Arsanis directors David McGirr and René Russo. In addition, on March 29, 2019, our board of directors elected Murray W. Stewart, M.D. to the board of directors. Our newly comprised board of directors may affect our business strategies and operating decisions that may have an adverse impact on our business, financial condition and results of operations.

We are an "emerging growth company," and the reduced disclosure requirements applicable to emerging growth companies may make our common stock 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.

We are a smaller reporting company. We cannot be certain if the reduced disclosure requirements applicable to smaller reporting companies will make our common stock less attractive to investors or otherwise limit our ability to raise additional funds.

We are currently a "smaller reporting company" under applicable securities regulations. A smaller reporting company is a company that has an aggregate market value of its voting stock held by non-affiliates, or public float, of less than $250 million (or, if the company has annual revenues of less than $100 million, a public float of less than $700 million or no public float) as of the last business day of its most recently completed second fiscal quarter. A smaller reporting company is able to provide simplified executive compensation disclosures in its filings, is exempt from the provisions of Section 404(b) of the Sarbanes-Oxley Act requiring that an independent registered public accounting firm provide an attestation report on the effectiveness of internal control over financial reporting, and has certain other reduced disclosure obligations in their SEC filings, including, among other things, only being required to provide two years of audited financial statements in annual reports. Reduced disclosure in our SEC filings due to our status as a smaller reporting company may make it harder for investors to analyze our results of operations and financial prospects.

In addition, we filed a universal shelf registration statement on Form S-3 with the SEC, which was declared effective on February 19, 2019 and pursuant to which we registered for sale up to $150 million of any combination of our common stock, preferred stock, debt securities, warrants and/or units from time to time and at prices and on terms that we may determine. Under SEC rules and regulations, we must meet certain requirements to use our Form S-3 registration statement to sell up to the full amount of $150 million of securities registered for sale under the Form S-3 registration statement. One such requirement is that the market value of our outstanding common stock held by non-affiliates, or public float, be at least $75 million as of a date within 60 days prior to the date on which the Form S-3 is filed (and within 60 days prior to the date of any Form 10-K filing thereafter by us, which is deemed a re-evaluation date). If we do not meet that requirement, then the aggregate market value of securities sold by us in a primary offering under our Form S-3 in any 12-month period is limited to an aggregate of one-third of our public float. SEC rules and regulations require that we periodically re-evaluate the value of our public float, and if, during the 60-day period prior the applicable re-evaluation date, our public float has not been equal to or greater than $75 million, we would become subject to the one-third of public float limitation described above. During the 60-day period prior to the date Arsanis's Annual Report on Form 10-K for the year ended December 31, 2018 was filed, which is our most recent re-evaluation date, our public float did not exceed $75 million. Following the Merger, during the 60-day period prior to March 31, 2019, our public float was greater than $75 million, and we are therefore not subject to the one-third of public float limitation, at least until our next re-evaluation date.

If our ability to utilize a Form S-3 registration statement becomes restricted under these rules, we could elect to raise capital pursuant to an exemption from registration under the Securities Act of 1933, as amended, or the Securities Act, or under a Form S-1 registration statement, but either of these alternatives would likely increase the cost of raising additional capital compared with the use of a Form S-3 registration statement. Furthermore, because of these limitations on primary securities offerings under a Form S-3 and the increased likelihood of greater costs and potential delays associated with the alternatives to using a Form S-3, the terms of any financing transaction that we are able to conduct may be less favorable or may cause us to be unable to obtain capital in a timely manner.

In addition, under current SEC rules and regulations, our common stock must be listed on a national securities exchange in order to utilize a Form S-3 registration statement (i) for a primary offering, if our public float is not at least $75 million as of a date within 60 days prior to the date on which the securities are sold under the Form S-3, or a re-evaluation date, whichever is later, and (ii) to register the resale of our securities by persons other than us. While our common stock is listed on The Nasdaq Capital Market, there can be no assurance that we will be able to maintain such listing.

If we fail to continue to meet the requirements for continued listing on The Nasdaq Capital Market, our common stock could be delisted from trading, which would decrease the liquidity of our common stock and ability to raise additional capital.

Our common stock is listed for quotation on the Nasdaq Capital Market. If we are unable to comply with Nasdaq's listing standards, Nasdaq may determine to delist our common stock from the Nasdaq Capital Market or other of Nasdaq's trading markets. If our common stock is delisted for any reason, it could reduce the value of our common stock and liquidity.

We will incur costs and demands upon management as a result of complying with the laws and regulations affecting public companies.

We will incur significant legal, accounting and other expenses that we did not incur as a private company, including costs associated with public company reporting requirements. We will also incur costs associated with corporate governance requirements, including requirements under the Sarbanes-Oxley Act, as well as rules implemented by the SEC and Nasdaq. These rules and regulations are expected to increase our legal and financial compliance costs and to make some activities more time consuming and costly. Our executive officers and other personnel will need to devote substantial time regarding operations as a public company and compliance with applicable laws and regulations. These rules and regulations may also make it difficult and expensive for us to obtain and maintain directors' and officers' liability insurance. As a result, it may be more difficult for us to attract and retain qualified individuals to serve on our board of directors or as our executive officers, which may adversely affect investor confidence in us and could cause our business or stock price to suffer.

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 rely, 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 Capital, Inc.

Future sales of shares by existing stockholders could cause our stock price to decline.

If our existing stockholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market after the lock-up and other legal restrictions on resale discussed below lapse, the trading price of our common stock could decline. As of March 13, 2019 following the completion of the Merger, approximately 3.5 million shares of our common stock were freely tradable without limitation to the extent permitted by the provisions of Rules 144 and 701 under the Securities Act.

The support agreements entered into by certain stockholders in connection with the Merger provide that the shares of our common stock, including, as applicable, shares received in the Merger and issuable upon exercise of certain options, subject to lock-up restrictions will be released from such restrictions as of the date which is 180 calendar days from the closing of the Merger (September 9, 2019). Upon expiration of such lockup restrictions, we expect up to an additional approximately 3.2 million shares of our common stock will be eligible for sale in the public market, approximately 0.2 million of which will be held by our directors, executive officers and other affiliates and will be subject to volume limitations under Rule 144 under the Securities Act. In addition, approximately 0.8 million shares of our common stock that were subject to outstanding stock options following the closing of the Merger on March 13, 2019 are or will become eligible for sale in the public market to the extent permitted by the provisions of various vesting agreements, the support agreements and Rules 144 and 701 under the Securities Act. 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.

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 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 will be required to furnish a report by our management on our internal control over financial reporting. 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. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, take steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that neither we, nor our independent registered public accounting firm once we are required to obtain an attestation report on internal control over financial reporting from such firm, will be able to conclude within the prescribed timeframe that our internal control over financial reporting is effective as required by Section 404. This could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

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 recently completed 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.

Special Note Regarding Forward-Looking Statements

These risk factors contain "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended that relate to future events or to our future operating or financial performance. Any forward-looking statement involves known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to differ materially from any future results, levels of activity, performance or achievements expressed or implied by such forward-looking statement. Forward-looking statements include statements, other than statements of historical fact, about, among other things:

- the progress, scope, cost, duration or results of our development activities, nonclinical studies and clinical trials of mavorixafor (X4P-001), X4P-002 and X4P-003 or any of our other product candidates or programs, such as the target indication(s) for development, the size, design, population, conduct, cost, objective or endpoints of any clinical trial, or the timing for initiation or completion of or availability of results from any clinical trial (including our planned trials for mavorixafor in Warts, Hypogammaglobulinemia, Infections, and Myelokathexis syndrome, severe congenital neutropenia and Waldenström macroglobulinemia) for submission or approval of any regulatory filing or for meeting with regulatory authorities;
- 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, any product candidates that we may develop, and any related restrictions, limitations, or warnings in the label of any approved product candidates;
- our future operations, financial position, revenues, costs, expenses, uses of cash, capital requirements or our need for additional financing; and
- our strategies, prospects, plans, expectations or objectives.

Words such as, but not limited to, "believe," "expect," "anticipate," "estimate," "forecast," "intend," "may," "plan," "potential," "predict," "project," "targets," "likely," "will," "would," "could," "should," "continue," "scheduled" and similar expressions or phrases, or the negative of those expressions or phrases, are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Although we believe that we have a reasonable basis for each forward-looking statement contained in this report, we caution you that these statements are based on our projections of the future that are subject to known and unknown risks and uncertainties and other important factors that may cause our actual results, level of activity, performance or achievements expressed or implied by any forward-looking statement to differ. These risks, uncertainties and other factors are described in greater detail under the caption "Risk Factors" in the filings that we make with the Securities and Exchange Commission. Also, these forward-looking statements represent our estimates and assumptions only as of the date of the document containing the applicable statement. As a result of the risks and uncertainties, the results or events indicated by the forward-looking statements may not occur. We caution you not to place undue reliance on any forward-looking statement.

You should read this report and the documents we have filed with the SEC that are incorporated by reference completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of the forward-looking statements in the foregoing documents by these cautionary statements. Unless required by law, we undertake no obligation to update or revise any forward-looking statements to reflect new information or future events or developments. Thus, you should not assume that our silence over time means that actual events are bearing out as expressed or implied in such forward-looking statements.