

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
Washington, D.C. 20549**

**FORM 10-Q**

(Mark One)

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

For the quarterly period ended September 30, 2019

or

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

For the transition period from \_\_\_\_\_ to \_\_\_\_\_

Commission File Number: 001-38295

**X4 PHARMACEUTICALS, INC.**

(Exact name of registrant as specified in its charter)

Delaware  
(State or other jurisdiction of  
incorporation or organization)

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

27-3181608  
(I.R.S. Employer  
Identification No.)

02139  
(Zip Code)

(857) 529-8300

(Registrant's telephone number, including area code)

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

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

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

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	<input type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input checked="" type="checkbox"/>	Smaller reporting company	<input checked="" type="checkbox"/>
		Emerging growth company	<input checked="" type="checkbox"/>

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

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

As of November 4, 2019, the registrant had 12,456,418 shares of common stock outstanding.

**X4 Pharmaceuticals, Inc.**

<a href="#">EXPLANATORY NOTE</a>	3
<a href="#">CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS</a>	3
<a href="#">PART I: FINANCIAL INFORMATION</a>	
Item 1. <a href="#">FINANCIAL STATEMENTS</a>	5
<a href="#">Condensed Consolidated Balance Sheets (unaudited) as of September 30, 2019 and December 31, 2018</a>	5
<a href="#">Condensed Consolidated Statements of Operations (unaudited) for the Three and Nine Months Ended September 30, 2019 and 2018</a>	6
<a href="#">Condensed Consolidated Statements of Comprehensive Loss (unaudited) for the Three and Nine Months Ended September 30, 2019 and 2018</a>	7
<a href="#">Condensed Consolidated Statements of Convertible Preferred Stock, Redeemable Common Stock and Stockholders' Equity (Deficit) (unaudited) for the Three and Nine Months Ended September 30, 2019 and 2018</a>	8
<a href="#">Condensed Consolidated Statements of Cash Flows (unaudited) for the Nine Months Ended September 30, 2019 and 2018</a>	10
<a href="#">Notes to Condensed Consolidated Financial Statements (unaudited)</a>	11
Item 2. <a href="#">MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS</a>	42
Item 3. <a href="#">QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK</a>	54
Item 4. <a href="#">CONTROLS AND PROCEDURES</a>	54
<a href="#">PART II: OTHER INFORMATION</a>	
Item 1. <a href="#">LEGAL PROCEEDINGS</a>	55
Item 1A. <a href="#">RISK FACTORS</a>	55
Item 2. <a href="#">UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS</a>	55
Item 3. <a href="#">DEFAULTS UPON SENIOR SECURITIES</a>	55
Item 4. <a href="#">MINE SAFETY DISCLOSURES</a>	55
Item 5. <a href="#">OTHER INFORMATION</a>	55
Item 6. <a href="#">EXHIBITS</a>	55
<a href="#">SIGNATURES</a>	57

## EXPLANATORY NOTE

On March 13, 2019, X4 Pharmaceuticals, Inc. (formerly Arsanis, Inc.), or the Company, completed its business combination 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 Therapeutics, Inc. (formerly X4 Pharmaceuticals, Inc.) and Artemis AC Corp., a Delaware corporation and a wholly-owned subsidiary of the Company, or the Merger Sub, pursuant to which, among other matters, Merger Sub merged with and into X4 Therapeutics, Inc., with X4 Therapeutics, Inc. continuing as a wholly-owned subsidiary of the Company and the surviving corporation of the merger, or the Merger. Following the Merger, on March 13, 2019, the Company effected a 1-for-6 reverse stock split of its common stock, or 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 Therapeutics, Inc., a clinical-stage biopharmaceutical company focused on the research, development and commercialization of novel therapeutics for the treatment of rare diseases.

Unless otherwise noted, all references to common stock share and per share amounts in this Quarterly Report on Form 10-Q have been retroactively adjusted to reflect the conversion of shares in the Merger based on an exchange ratio of 0.5702 and the Reverse Stock Split. As used herein, the words "the Company," "we," "us," and "our" refer to, for periods following the Merger, X4 Pharmaceuticals, Inc. (formerly Arsanis, Inc.), together with its direct and indirect subsidiaries, and for periods prior to the Merger, X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals, Inc.), and its direct and indirect subsidiaries, as applicable. In addition, the word "Arsanis" refers to the Company prior to the completion of the Merger, and the word "X4" refers to X4 Therapeutics, Inc.

### CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act, that relate to future events or to our future operations or financial performance. These statements may be identified by such forward-looking terminology as "may," "should," "expects," "intends," "plans," "anticipates," "believes," "estimates," "predicts," "potential," "continue" or the negative of these terms or other comparable terminology. Our forward-looking statements are based on a series of expectations, assumptions, estimates and projections about our company, are not guarantees of future results or performance and involve substantial risks and uncertainty. We may not actually achieve the plans, intentions or expectations disclosed in these forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in these forward-looking statements. These risks, uncertainties and other factors are described in greater detail under the caption "Risk Factors" contained in our Annual Report on Form 10-K for the fiscal year ended December 31, 2018, as updated by our Current Report on Form 8-K filed on April 11, 2019 and our other filings that we make with the Securities and Exchange Commission, or SEC.

Our business and our forward-looking statements involve substantial known and unknown risks and uncertainties, including the risks and uncertainties inherent in our statements regarding:

- 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, or WHIM, syndrome, severe congenital neutropenia, or SCN, and Waldenström macroglobulinemia, or WM, 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 plans to research, develop, manufacture and commercialize our product candidates;
- the timing of our regulatory filings for our product candidates, along with regulatory developments in the United States and other foreign countries;
- our commercialization, marketing and manufacturing capabilities and strategy;

**X4 PHARMACEUTICALS, INC.**

- our ability to attract and retain qualified employees and key personnel;
- our competitive position;
- our expectations regarding our ability to obtain and maintain intellectual property protection;
- our estimates and expectations regarding future operations, financial position, revenues, costs, expenses, uses of cash, capital requirements or our need for additional financing;
- our ability to raise capital; and
- our strategies, prospects, plans, expectations or objectives.

In addition, any forward-looking statement in this Quarterly Report represents our views only as of the date of this Quarterly Report and should not be relied upon as representing our views as of any subsequent date. We anticipate that subsequent events and developments may cause our views to change. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, levels of activity, performance or achievements. Except as required by law, we are under no duty to update or revise any of the forward-looking statements, whether because of new information, future events or otherwise, after the date of this Quarterly Report.

**PART I FINANCIAL INFORMATION**

**Item 1. FINANCIAL STATEMENTS.**

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

	<u>September 30, 2019</u>	<u>December 31, 2018</u>
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 76,251	\$ 8,134
Research and development incentive receivable	1,730	—
Prepaid expenses and other current assets	1,234	1,205
Total current assets	79,215	9,339
Property and equipment, net	348	241
Goodwill	27,109	—
Right-of-use assets	2,110	—
Restricted cash	755	364
Total assets	<u>\$ 109,537</u>	<u>\$ 9,944</u>
<b>Liabilities, Convertible Preferred Stock, Redeemable Common Stock and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable	\$ 1,704	\$ 2,969
Accrued expenses	5,697	3,251
Current portion of lease liability	878	—
Current portion of long-term debt, net of discount	—	1,687
Total current liabilities	8,279	7,907
Preferred stock warrant liability	—	4,947
Long-term debt, including accretion, net of discount and current portion	19,986	8,145
Deferred rent	—	417
Lease liability	2,147	—
Other liabilities	18	205
Total liabilities	30,430	21,621
Commitments and contingencies (Note 10)		
Convertible preferred stock (Series Seed, A and B), \$0.001 par value; 10,000,000 and 59,413,523 shares authorized as of September 30, 2019 and December 31, 2018, respectively; 0 and 40,079,567 shares issued and outstanding as of September 30, 2019 and December 31, 2018, respectively	—	64,675
Redeemable common stock, \$0.001 par value; 0 and 107,364 shares issued and outstanding as of September 30, 2019 and December 31, 2018, respectively	—	734
Stockholders' equity (deficit):		
Common stock, \$0.001 par value. 33,333,333 and 11,070,776 shares authorized as of September 30, 2019 and December 31, 2018, respectively; 12,436,205 and 351,652 shares issued and outstanding as of September 30, 2019 and December 31, 2018, respectively	12	—
Additional paid-in capital	200,421	2,151
Accumulated other comprehensive income (loss)	(119)	—
Accumulated deficit	(121,207)	(79,237)
Total stockholders' equity (deficit)	79,107	(77,086)
Total liabilities, convertible preferred stock, redeemable common stock and stockholders' equity (deficit)	<u>\$ 109,537</u>	<u>\$ 9,944</u>

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

**X4 PHARMACEUTICALS, INC.**  
**CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS**

(In thousands, except per share amounts)

(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
<b>Operating expenses:</b>				
Research and development	\$ 8,589	\$ 6,158	\$ 23,098	\$ 15,657
General and administrative	4,383	2,387	13,726	5,374
Loss on transfer of non-financial assets (Note 16)	4,004	—	4,004	—
Total operating expenses	<u>16,976</u>	<u>8,545</u>	<u>40,828</u>	<u>21,031</u>
Loss from operations	(16,976)	(8,545)	(40,828)	(21,031)
<b>Other income (expense):</b>				
Interest income	464	51	927	187
Interest expense	(688)	(148)	(1,599)	(484)
Change in fair value of preferred stock warrant liability	—	45	(288)	(264)
Change in fair value of derivative liability	—	(5)	183	(411)
Loss on extinguishment of debt	(566)	—	(566)	—
Other income (expense)	52	—	201	—
Total other income (expense), net	<u>(738)</u>	<u>(57)</u>	<u>(1,142)</u>	<u>(972)</u>
Net loss	(17,714)	(8,602)	(41,970)	(22,003)
Accruing dividends on Series A convertible preferred stock	—	(756)	(592)	(2,244)
Adjustment to accumulated deficit in connection with repurchase of Series Seed convertible preferred stock	—	—	—	(22)
Net loss attributable to common stockholders	<u>\$ (17,714)</u>	<u>\$ (9,358)</u>	<u>\$ (42,562)</u>	<u>\$ (24,269)</u>
Net loss per share attributable to common stockholders—basic and diluted	<u>\$ (1.22)</u>	<u>\$ (20.39)</u>	<u>\$ (4.31)</u>	<u>\$ (52.92)</u>
Weighted average common shares outstanding—basic and diluted	<u>14,562</u>	<u>459</u>	<u>9,866</u>	<u>459</u>

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

**X4 PHARMACEUTICALS, INC.**  
**CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS**

(In thousands)

(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Net loss	\$ (17,714)	\$ (8,602)	\$ (41,970)	\$ (22,003)
Other comprehensive loss				
Currency translation adjustments	(77)	—	(119)	—
Total comprehensive loss	\$ (17,791)	\$ (8,602)	\$ (42,089)	\$ (22,003)

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

X4 PHARMACEUTICALS, INC.

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

(In thousands, except share amounts)

(Unaudited)

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

X4 PHARMACEUTICALS, INC.

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

(In thousands, except share amounts)

(Unaudited)

	Series Seed, A and B Convertible Preferred		Redeemable Common Stock		Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' (Deficit)
	Shares	Amount	Shares	Amount	Shares	Amount				
<b>Balance at December 31, 2017</b>	38,018,968	\$ 60,903	107,364	\$ 734	350,607	—	\$ 1,385	—	\$ (45,930)	\$ (44,545)
Repurchase of Series Seed convertible preferred stock, net of issuance costs of \$11	(598,975)	(517)							(22)	(22)
Stock-based compensation							128			128
Net loss									(7,367)	(7,367)
<b>Balance at March 31, 2018</b>	37,419,993	60,386	107,364	734	350,607	—	1,513	—	(53,319)	(51,806)
Exercise of stock options					1,045		7			7
Stock-based compensation expense							145			145
Net loss									(6,034)	(6,034)
<b>Balance at September 30, 2018</b>	37,419,993	60,386	107,364	734	351,652	—	1,665	—	(59,353)	(57,688)
Issuance of Series B convertible preferred stock, net of issuance costs of \$539	2,659,574	4,289								—
Stock-based compensation expense							198			198
Net loss									(8,602)	(8,602)
<b>Balance at September 30, 2018</b>	40,079,567	\$ 64,675	107,364	\$ 734	351,652	\$ —	\$ 1,863	\$ —	\$ (67,955)	\$ (66,092)

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

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

	Nine Months Ended September 30,	
	2019	2018
<b>Cash flows from operating activities:</b>		
Net loss	\$ (41,970)	\$ (22,003)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	1,386	471
Depreciation and amortization expense	71	77
Loss on transfer of non-financial assets (Note 16)	4,004	—
Non-cash lease expense	416	—
Accretion of debt discount	584	80
Loss on extinguishment of debt	566	—
Change in fair value of preferred stock warrant liability	288	264
Change in fair value of derivative liability	(183)	411
<b>Changes in operating assets and liabilities:</b>		
Prepaid expenses, other current assets and research and development incentive receivable	290	631
Accounts payable	(3,131)	209
Accrued expenses	(595)	1,174
Lease liabilities	(540)	—
Net cash used in operating activities	(38,814)	(18,686)
<b>Cash flows from investing activities:</b>		
Cash, cash equivalents and restricted cash acquired in connection with the Merger	26,406	—
Proceeds from transfer of non-financial assets	896	—
Acquisition of property, equipment and intangible assets	(91)	—
Net cash provided by investing activities	27,211	—
<b>Cash flows from financing activities:</b>		
Proceeds from exercise of stock options and warrants	605	7
Proceeds from borrowings under loan and security agreements, net of issuance costs	9,849	—
Proceeds from issuance of Series B convertible preferred stock, net of issuance costs	—	4,461
Repurchase of Series Seed convertible preferred stock	—	(1,126)
Repayments of borrowings under loan and security agreement	(9,368)	(1,500)
Proceeds from sale of common stock and warrants, net of issuance costs	79,291	—
Net cash provided by financing activities	80,377	1,842
Effect of exchange rate changes on cash, cash equivalents and restricted cash	(266)	—
<b>Net increase (decrease) in cash, cash equivalents and restricted cash</b>	<b>68,508</b>	<b>(16,844)</b>
Cash, cash equivalents and restricted cash at beginning of period	8,498	27,048
Cash, cash equivalents and restricted cash at end of period	\$ 77,006	\$ 10,204
<b>Supplemental disclosure of non-cash investing and financing activities:</b>		
Acquisition of property, equipment and intangible assets included in accounts payable	\$ 38	\$ —
Conversion of convertible preferred stock into common stock	\$ 64,675	\$ —
Conversion of redeemable common stock into common stock	\$ 734	\$ —
Conversion of convertible preferred stock warrants into common stock warrants	\$ 5,235	\$ —
Fair value of net assets acquired in the Merger	\$ 46,358	\$ —

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

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

**1. Nature of the Business and Basis of Presentation**

X4 Pharmaceuticals, Inc. (formerly Arsanis, Inc.), together with its subsidiaries (the “Company”), is a clinical-stage biotechnology company focused on the research, development and commercialization of novel therapeutics for the treatment of rare diseases. The Company’s lead product candidate, mavorixafor (X4P-001), is a potential first-in-class, once-daily, oral inhibitor of CXCR4 and is currently in a Phase 3 clinical trial for the treatment of WHIM syndrome, a rare, inherited, primary immunodeficiency disease caused by genetic mutations in the CXCR4 receptor gene. The Company is headquartered in Cambridge, Massachusetts.

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including, but not limited to, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with governmental regulations and the ability to secure additional capital to fund operations. Drug candidates currently under development will require extensive preclinical and clinical testing and regulatory approval prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel, and infrastructure and extensive compliance-reporting capabilities. Even if the Company’s drug development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

***Merger with Arsanis***

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

The transaction was accounted for as a reverse merger in accordance with accounting principles generally accepted in the United States of America (“GAAP”). Under this method of accounting, X4 was deemed to be the accounting acquirer for financial reporting purposes. This determination was primarily based on the facts that, immediately following the Merger: (i) the Company’s stockholders own a substantial majority of the voting rights in the combined organization, (ii) the Company designated a majority of the members of the initial board of directors of the combined organization and (iii) the Company’s senior management hold all key positions in the senior management of the combined organization. Accordingly, for accounting purposes, the business combination was treated as the equivalent of X4 issuing stock to acquire the net assets of Arsanis. As a result, as of the closing date of the Merger, the net assets of Arsanis were recorded at their acquisition-date fair values in the financial statements of the Company and the reported operating results prior to the business combination are those of the Company. In addition, transaction costs incurred by the Company in connection with the business combination have been expensed as incurred.

On March 13, 2019, Arsanis, X4 and Merger Sub completed the Merger pursuant to the terms of the Merger Agreement. Pursuant to the terms of the Merger Agreement, each outstanding share of X4’s common stock and preferred stock was exchanged for 0.5702 shares of Arsanis’s common stock (the “Exchange Ratio”). In addition, all outstanding options exercisable for common stock and warrants exercisable for convertible preferred stock of X4 became options and warrants exercisable for the same number of shares of common stock of Arsanis multiplied by the Exchange Ratio. In connection with the Merger, X4 changed its name to X4 Therapeutics, Inc. Following the closing of the Merger, X4 Therapeutics, Inc. became a wholly-owned subsidiary of the Company, which changed its name to X4 Pharmaceuticals, Inc. As used herein, the words “the Company” refers to, for periods following the Merger, X4 Pharmaceuticals, Inc. (formerly Arsanis, Inc.), together with its direct and indirect subsidiaries, and for periods prior to the Merger, X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals, Inc.), and its direct and indirect subsidiaries, as applicable.

Immediately following the Merger, stockholders of X4 owned approximately 64% of the combined organization’s outstanding common stock. On March 14, 2019, the combined organization’s common stock began trading on The Nasdaq Capital Market under the ticker symbol “XFOR.”

***Principles of Consolidation***— The condensed consolidated financial statements include the accounts of the Company and its wholly-owned subsidiaries, including X4 Pharmaceuticals (Austria) GmbH, which is incorporated in Vienna, Austria and was

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

formerly named Arsanis Biosciences GmbH (“X4 GmbH”), and X4 Therapeutics, Inc. All significant intercompany accounts and transactions have been eliminated.

**Reverse Stock Split**— On March 13, 2019, immediately following the closing of the Merger, the Company effected a 1-for-6 reverse stock split of its common stock (the “Reverse Stock Split”). Accordingly, all share and per share amounts for all periods presented in the accompanying condensed consolidated financial statements and notes thereto have been adjusted retroactively, where applicable, to reflect the Reverse Stock Split. No fractional shares were issued in connection with the Reverse Stock Split. Unless otherwise noted, all references to common stock share and per share amounts have also been adjusted to reflect the exchange ratio of 0.5702.

**Going Concern Assessment**— In accordance with Accounting Standards Update (“ASU”) No. 2014-15, *Disclosure of Uncertainties about an Entity’s Ability to Continue as a Going Concern (Subtopic 205-40)*, the Company has evaluated whether there are certain conditions and events, considered in the aggregate, that raise substantial doubt about the Company’s ability to continue as a going concern within one year after the date that the consolidated financial statements are issued. As of November 7, 2019, the issuance date of these condensed consolidated financial statements, the Company expects that its cash and cash equivalents will be sufficient to fund its forecasted operating expenses, capital expenditure requirements and debt service payments for at least the next twelve months from the date of these financial statements. The future viability of the Company beyond that point is dependent on its ability to raise additional capital to finance its operations.

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

## **2. Summary of Significant Accounting Policies**

**Unaudited Interim Financial Statements**— The condensed balance sheet at December 31, 2018 was derived from audited financial statements but does not include all disclosures required by GAAP. The accompanying condensed consolidated financial statements are unaudited. The accompanying unaudited interim consolidated financial statements have been prepared by the Company pursuant to the rules and regulations of the Securities and Exchange Commission (the “SEC”) for interim financial statements. Accordingly, certain information and footnote disclosures normally included in financial statements prepared in accordance with GAAP have been condensed or omitted pursuant to such rules and regulations. However, the Company believes that the disclosures are adequate to make the information presented not misleading. These unaudited condensed consolidated financial statements should be read in conjunction with the Company’s audited financial statements and the notes thereto for the year ended December 31, 2018. In the opinion of management, all adjustments, consisting only of normal recurring adjustments as necessary, for the fair statement of the Company’s condensed financial position, condensed results of its operations and cash flows have been made. The results of operations for the three and nine months ended September 30, 2019 are not necessarily indicative of the results of operations that may be expected for the year ending December 31, 2019.

**Use of Estimates**— The preparation of the Company’s condensed consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the condensed consolidated financial statements, and the reported amounts of expenses during the reporting period. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to, the accrual of research and development expenses, the valuation of intangible assets acquired in business combinations, the valuations of common stock prior to the Merger, the valuation of stock options, preferred stock warrants (and the resulting preferred stock warrant liability), derivative instruments (and the resulting derivative liabilities), and the preferred stock repurchase liability, valuation of lease liabilities and the constraint of variable consideration from revenue transactions. The Company bases its estimates on historical experience, known trends and other market-specific or other relevant factors that it believes to be reasonable under the circumstances. On an ongoing basis, management evaluates its estimates when there are changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results could differ from those estimates.

**Foreign Currency and Currency Translation**—For the Company’s subsidiaries that transact in a functional currency other than the U.S. dollar, assets and liabilities are translated at current exchange rates as of the balance sheet date while income and

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

expenses are translated at the average exchange rates for the period. Adjustments resulting from the translation of the financial statements of the Company's foreign operations into U.S. dollars are excluded from the determination of net loss and are recorded in accumulated other comprehensive loss, a component of stockholders' equity (deficit).

**Concentrations of Credit Risk and Significant Suppliers**— Financial instruments that potentially subject the Company to concentrations of credit risk consist principally of cash and cash equivalents and research and development incentive receivables. The Company generally maintains cash balances in various operating accounts at financial institutions that management believes to be of high credit quality in amounts that may exceed federally insured limits. The Company has not experienced losses related to its cash and cash equivalents.

The Company is dependent on third-party manufacturers to supply products for research and development activities in its programs. The Company relies and expects to continue to rely on a small number of manufacturers to supply it with its requirements for the active pharmaceutical ingredients and formulated drugs related to these programs. These programs could be adversely affected by a significant interruption in these manufacturing services or in the supply of active pharmaceutical ingredients and formulated drugs.

**Cash and Cash Equivalents**— The Company considers all highly liquid investments with maturities of three months or less at the date of purchase to be cash equivalents. Cash equivalents consisted of money market funds as of September 30, 2019 and December 31, 2018.

**Restricted Cash**—

<i>(in thousands)</i>	As of September 30, 2019	As of December 31, 2018
Letter of credit security: Cambridge lease <sup>(a)</sup>	\$ 264	\$ 264
Letter of credit security: Waltham lease <sup>(b)</sup>	250	—
Letter of credit security: Vienna Austria lease <sup>(c)</sup>	91	—
Corporate credit card collateral <sup>(d)</sup>	150	100
<b>Total restricted cash (non-current)</b>	<b>\$ 755</b>	<b>\$ 364</b>

- (a) In connection with the Company's lease agreement for its facility in Cambridge, Massachusetts, the Company maintains a letter of credit of \$264 thousand, which is secured by restricted cash, for the benefit of the landlord.
- (b) In connection with the Company's lease agreement for a facility in Waltham, Massachusetts, the Company maintains a letter of credit of \$250 thousand, which is secured by restricted cash for the benefit of the landlord.
- (c) In connection with the Company's lease agreement for its laboratory and office facility in Vienna, Austria, the Company maintains a letter of credit of \$91 thousand, which is secured by restricted cash, for the benefit of the landlord.
- (d) As of September 30, 2019, and December 31, 2018, the Company was required to maintain a separate cash balance of \$50 thousand and \$100 thousand, respectively, to collateralize corporate credit cards with a bank.

In accordance with the Company's Amended and Restated Loan Agreement with Hercules Capital, Inc. ("Hercules") as further described in Note 8, the Company is required to maintain cash in an account accessible by the lender in an amount not less than 125% of the outstanding loan balance, or if the Company's consolidated cash is lower than this amount, all of the Company's cash other than \$2.5 million. As of September 30, 2019, the Company maintained \$25 million in an account accessible by the lender in accordance with the terms of Amended and Restated Loan Agreement.

The following table provides a reconciliation of cash, cash equivalents, and restricted cash reported within the condensed consolidated balance sheets to the sum to the total of amounts shown in the Company's condensed consolidated statement of cash flows as of September 30, 2019, December 31, 2018, September 30, 2018 and December 31, 2017:

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

<i>(in thousands)</i>	September 30, 2019	December 31, 2018	September 30, 2018	December 31, 2017
Cash and cash equivalents	\$ 76,251	\$ 8,134	\$ 9,840	\$ 26,684
Restricted cash, non-current	755	364	364	364
Total cash, cash equivalents and restricted cash	<u>\$ 77,006</u>	<u>\$ 8,498</u>	<u>\$ 10,204</u>	<u>\$ 27,048</u>

**Property and Equipment**—Property and equipment are stated at cost less accumulated depreciation and amortization. Depreciation and amortization expense is recognized using the straight-line method over the estimated useful life of each asset, as follows:

	Estimated Useful Life
Office furniture	3 years
Computer equipment	3 years
Laboratory equipment	3 to 10 years
Leasehold improvements	Shorter of lease term or 10 years

Estimated useful lives are periodically assessed to determine if changes are appropriate. Maintenance and repairs are charged to expense as incurred. When assets are retired or otherwise disposed of, the cost of these assets and related accumulated depreciation or amortization are eliminated from the condensed consolidated balance sheet and any resulting gains or losses are included in the condensed consolidated statements of operations and comprehensive loss in the period of disposal. Costs for capital assets not yet placed into service are capitalized as construction-in-progress and depreciated once placed into service.

**Right-of-Use Assets and Leases**— Effective January 1, 2019, the Company adopted Accounting Standards Codification (“ASC”), Topic 842, *Leases* (“ASC 842”), using the modified retrospective approach through a cumulative-effect adjustment and utilizing the effective date as its date of initial application, with prior periods unchanged and presented in accordance with the guidance in Topic 840, *Leases* (“ASC 840”).

At the inception of an arrangement, the Company determines whether the arrangement contains a lease based on the unique facts and circumstances present. Leases with a non-cancellable term greater than one year are recognized on the balance sheet as right-of-use assets, lease liabilities and long-term lease liabilities. The Company has elected not to recognize on the balance sheet leases with terms of one year or less. Options to renew a lease are not included in the Company’s initial lease term assessment unless there is reasonable certainty that the Company will renew the lease. If a lease is cancellable without penalty, the Company excludes from the lease term periods following the cancellation notice period unless it is reasonably certain that the Company will not cancel the lease.

Operating lease liabilities and their corresponding right-of-use assets are recorded based on the present value of lease payments over the expected remaining lease term. Certain adjustments to the right-of-use operating asset may be required for items such as incentives received or accrued rent. The interest rate implicit in lease contracts is typically not readily determinable. As a result, the Company utilizes its incremental borrowing rates, which are the rates it incurs to borrow on a collateralized basis over a similar term an amount equal to the lease payments in a similar economic environment.

In accordance with the guidance in ASC 842, components of a lease are split into lease components and non-lease components. A policy election is available pursuant to which an entity may elect to not separate lease and non-lease components. Rather, each lease component and the related non-lease components are accounted for together as a single component. For new and amended leases beginning in 2019 and after, the Company has elected to account for the lease and non-lease components as a combined lease component for its office and laboratory building leases.

**Impairment of Long-Lived Assets**— Long-lived assets consist of property and equipment. Long-lived assets to be held and used are tested for recoverability whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable. Factors that the Company considers in deciding when to perform an impairment review include significant underperformance of the business in relation to expectations, significant negative industry or economic trends and significant changes or planned changes in the use of the assets. If an impairment review is performed to evaluate a long-lived asset group for recoverability, the Company compares forecasts of undiscounted cash flows expected to result from the use and eventual disposition of the long-lived asset group to its carrying value. An impairment loss would be recognized in loss from operations when estimated undiscounted future cash flows expected to result from the use of an asset group are less

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

than its carrying amount. The impairment loss would be based on the excess of the carrying value of the impaired asset group over its fair value. To date, the Company has not recorded any material impairment losses on long-lived assets.

**Goodwill**— Business combinations are accounted for under the acquisition method. The total purchase price of an acquisition is allocated to the underlying identifiable net assets, based on their respective estimated fair values as of the acquisition date. Determining the fair value of assets acquired and liabilities assumed requires management's judgment and often involves the use of significant estimates and assumptions, including assumptions with respect to future cash inflows and outflows, probabilities of success, discount rates, and asset lives, among other items. Assets acquired and liabilities assumed are recorded at their estimated fair values. The excess of the purchase price over the estimated fair values of the net assets acquired is recorded as goodwill.

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

The Company assesses qualitative factors to determine whether the existence of events or circumstances would indicate that it is more likely than not that the fair value of the reporting unit was less than its carrying amount. If after assessing the totality of events or circumstances, the Company were to determine that it is more likely than not that the fair value of the reporting unit is less than its carrying amount, then the Company would perform a quantitative impairment test.

The Company compares the fair value of the reporting unit to its carrying value. If the fair value of the reporting unit exceeds the carrying value of the net assets, goodwill is not impaired, and no further testing is required. If the fair value of the reporting unit is less than the carrying value, the Company measures the amount of impairment loss, if any, as the excess of the carrying value over the fair value of the reporting unit. The Company has determined there were no indicators of goodwill impairment as of September 30, 2019.

**Intangible Assets**— In connection with the Merger, the Company acquired certain IPR&D assets, which were classified as indefinite-lived intangible assets. Acquired IPR&D represents the fair value assigned to research and development assets that the Company acquires and have not been completed at the acquisition date. The fair value of IPR&D acquired in a business combination is recorded on the Company's condensed consolidated balance sheets at the acquisition-date fair value and is determined by estimating the costs to develop the technology into commercially viable products, estimating the resulting revenue from the projects, and discounting the projected net cash flows to present value. IPR&D is not amortized, but rather is reviewed for impairment on an annual basis or more frequently if indicators of impairment are present, until the project is completed, abandoned or transferred to a third party.

The projected discounted cash flow models used to estimate the Company's IPR&D reflect significant assumptions regarding the estimates a market participant would make in order to evaluate a drug development asset, including the following:

- Probability of successfully completing clinical trials and obtaining regulatory approval;
- Market size, market growth projections, and market share;
- Estimates regarding the timing of and the expected costs to advance the Company's clinical programs to commercialization;
- Estimates of future cash flows from potential product sales; and
- A discount rate reflecting the Company's weighted average cost of capital and specific risk inherent in the underlying assets

During the quarter ended September 30, 2019, the Company entered into an out-licensing arrangement with a third party that transferred the rights to develop and commercialize one of the programs underlying an IPR&D intangible asset. In addition, the Company entered into amended out-licensing option agreements with a third party who had previously entered into an option agreement with Arsanis to license the rights to develop and commercialize two other programs underlying the IPR&D intangible assets. Following the amendment to these option agreements, the options were exercised by the third party and the in-process research and development programs were out-licensed to the third party. As of September 30, 2019, all programs' underlying IPR&D intangible assets acquired in the Merger were transferred to these third parties and the Company has no

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

continuing involvement in any ongoing research and development activities associated with the programs. As a result of the transfer of the IPR&D projects to third parties, the Company derecognized the IPR&D intangibles asset through a charge to "loss on transfer of non-financial assets" during the third quarter of 2019. See Note 16.

**Deferred Rent**— The Company's lease agreements include payment escalations and lease incentives (including a leasehold improvement tenant allowance). For periods prior to January 1, 2019, these payments were accrued or deferred as appropriate such that rent expense was recognized on a straight-line basis over the respective lease terms. Effective January 1, 2019, upon the adoption of ASC 842, deferred rent was reclassified as a reduction to the applicable right-of-use asset as further described in Note 9.

**Fair Value Measurements**— Certain assets and liabilities of the Company are carried at fair value under GAAP. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

- Level 1—Quoted prices in active markets for identical assets or liabilities.
- Level 2—Observable inputs (other than Level 1 quoted prices), such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities, or other inputs that are observable or can be corroborated by observable market data.
- Level 3—Unobservable inputs that are supported by little or no market activity that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

Prior to the Merger, the Company's preferred stock warrant liability, derivative liability and preferred stock repurchase liability were carried at fair value, determined according to Level 3 inputs in the fair value hierarchy described above (see Note 5). The Company's cash equivalents, consisting of money market funds invested in U.S. Treasury securities, are carried at fair value, determined based on Level 2 inputs in the fair value hierarchy described above. The carrying values of the Company's accounts payable and accrued expenses approximate their fair values due to the short-term nature of these liabilities. The carrying value of the Company's outstanding loan and security agreement (the "Hercules Loan Agreement") with Hercules approximates its fair value at September 30, 2019 because the debt bears interest at a variable market rate and the Company's credit risk has not materially changed since the inception of the agreement.

**Segment Information**— The Company manages its operations as a single operating segment for the purposes of assessing performance and making operating decisions. The Company's focus is on the research, development and commercialization of novel therapeutics for the treatment of rare diseases.

**Revenue Recognition**— Effective January 1, 2018, the Company adopted ASC Topic 606, *Revenue from Contracts with Customers* ("ASC 606"), as amended, using the modified retrospective transition method. The modified retrospective method requires that the cumulative effect of initially applying ASC 606 be recognized as an adjustment to the opening balance of retained earnings or accumulated deficit of the annual period that includes the date of initial application. The Company had no arrangements that were in the scope of ASC 606 on January 1, 2018 and thus there was no impact to the condensed consolidated financial statements as a result of the adoption. This standard applies to all contracts with customers, except for contracts that are within the scope of other standards, such as collaboration arrangements and leases.

The Company's revenues are generated primarily through research, development and commercialization agreements. The terms of these agreements may contain multiple promised goods and services, which may include (i) licenses, or options to obtain licenses, to the Company's technology, and (ii) in certain cases, services in connection with the manufacturing of preclinical and clinical materials. Payments to the Company under these arrangements typically include one or more of the following: non-refundable, upfront license fees; option exercise fees; milestone payments; payments for clinical and commercial product supply, and royalties on future product sales.

The Company analyzes its arrangements to assess whether such arrangements involve joint operating activities performed by parties that are both active participants in the activities and exposed to significant risks and rewards dependent on the commercial success of such activities and are therefore within the scope of ASC Topic 808, *Collaborative Arrangements*

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

(“ASC 808”). This assessment is performed throughout the life of the arrangement based on changes in the responsibilities of all parties in the arrangement. For collaboration arrangements that are deemed to be within the scope of ASC 808, the Company first determines which elements of the collaboration are deemed to be within the scope of ASC 808 and those that are more reflective of a vendor-customer relationship and, therefore, within the scope of ASC 606. The Company’s policy is generally to recognize amounts received from collaborators in connection with joint operating activities that are within the scope of ASC 808 as a reduction in research and development expense. To date, there have been no transactions within the scope of ASC 808.

Under ASC 606, the Company recognizes revenue when its customers obtain control of promised goods or services, in an amount that reflects the consideration which the Company determines it expects to receive in exchange for those goods or services. To determine revenue recognition for arrangements that the Company determines are within the scope of ASC 606, the Company performs the following five steps: (1) identify the contract(s) with a customer; (2) identify the performance obligation(s) in the contract; (3) determine the transaction price; (4) allocate the transaction price to the performance obligation(s) in the contract; and (5) recognize revenue when (or as) the Company satisfies its performance obligation(s).

As part of the accounting for these arrangements, the Company must make significant judgments, including identifying performance obligations in the contract, estimating the amount of variable consideration to include in the transaction price and; allocating the transaction price to each performance obligation.

Once a contract is determined to be within the scope of ASC 606, the Company assesses the goods or services promised within the contract and determines those that are performance obligations. Arrangements that include rights to additional goods or services that are exercisable at a customer’s discretion are generally considered options. The Company assesses if these options provide a material right to the customer and if so, they are considered performance obligations.

The Company assesses whether each promised good or service is distinct for the purpose of identifying the performance obligations in the contract. This assessment involves subjective determinations and requires management to make judgments about the individual promised goods or services and whether such are separable from the other aspects of the contractual relationship. Promised goods and services are considered distinct provided that:

- i. the customer can benefit from the good or service either on its own or together with other resources that are readily available to the customer (that is, the good or service is capable of being distinct) and
- ii. the entity’s promise to transfer the good or service to the customer is separately identifiable from other promises in the contract (that is, the promise to transfer the good or service is distinct within the context of the contract).

In assessing whether a promised good or service is distinct, the Company considers factors such as the research, manufacturing and commercialization capabilities of the customer and the availability of the associated expertise in the general marketplace. The Company also considers the intended benefit of the contract in assessing whether a promised good or service is separately identifiable from other promises in the contract. If a promised good or service is not distinct, an entity is required to combine that good or service with other promised goods or services until it identifies a bundle of goods or services that is distinct.

The transaction price is then determined and allocated to the identified performance obligations in proportion to their standalone selling prices (“SSP”) on a relative SSP basis. SSP is determined at contract inception and is not updated to reflect changes between contract inception and when the performance obligations are satisfied. Determining the SSP for performance obligations requires significant judgment. In developing the SSP for a performance obligation, the Company considers applicable market conditions and relevant entity-specific factors, including factors that were contemplated in negotiating the agreement with the customer and estimated costs. The Company validates the SSP for performance obligations by evaluating whether changes in the key assumptions used to determine the SSP will have a significant effect on the allocation of arrangement consideration between multiple performance obligations.

If the consideration promised in a contract includes a variable amount, the Company estimates the amount of consideration to which it will be entitled in exchange for transferring the promised goods or services to a customer. The Company determines the amount of variable consideration by using either the expected value method or the most-likely-amount method. The Company includes the unconstrained amount of estimated variable consideration in the transaction price. The amount included in the transaction price reflects the amount for which it is probable that a significant reversal of cumulative revenue recognized will not occur. At the end of each subsequent reporting period, the Company re-evaluates the estimated variable consideration included in the transaction price and any related constraint, and if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis in the period of adjustment.

In determining the transaction price, the Company adjusts consideration for the effects of the time value of money if the timing of payments provides the Company with a significant benefit of financing. The Company does not assess whether a contract has

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

a significant financing component if the expectation at contract inception is such that the period between payment by the licensees and the transfer of the promised goods or services to the licensees will be one year or less. The Company assessed each of its revenue generating arrangements in order to determine whether a significant financing component exists and concluded that a significant financing component does not exist in any of its arrangements.

The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) each performance obligation is satisfied at a point in time or over time, and if over time based on the use of an output or input method.

At the inception of each arrangement that includes non-refundable payments for contingent milestones, including preclinical research and development, clinical development and regulatory, the Company evaluates whether the milestones are considered probable of being achieved and estimates the amount to be included in the transaction price using the most-likely-amount method. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the control of the Company or the licensee, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. At the end of each reporting period, the Company re-evaluates the probability of the achievement of contingent milestones and the likelihood of a significant reversal of such milestone revenue, and if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect licensing revenue in the period of adjustment. This quarterly assessment may result in the recognition of revenue related to a contingent milestone payment before the milestone event has been achieved.

**Research and Development Programs**— Proceeds under the research and development incentive program from the Austrian government are recognized as other income in an amount equal to the qualifying expenses incurred in each period multiplied by the applicable reimbursement percentage. Incentive income recognized upon incurring qualifying expenses in advance of receipt of proceeds from research and development incentives is recorded in the condensed consolidated balance sheet as research and development incentive receivable.

**Research and Development Costs**— Costs associated with internal research and development and external research and development services, including drug development and preclinical studies, are expensed as incurred. Research and development expenses include costs for salaries, employee benefits, subcontractors, facility-related expenses, depreciation and amortization, stock-based compensation, third-party license fees, laboratory supplies, and external costs of outside vendors engaged to conduct discovery, preclinical and clinical development activities and clinical trials as well as to manufacture clinical trial materials, and other costs. The Company recognizes external research and development costs based on an evaluation of the progress to completion of specific tasks using information provided to the Company by its service providers.

Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. Such prepaid expenses are recognized as an expense when the goods have been delivered or the related services have been performed, or when it is no longer expected that the goods will be delivered or the services rendered.

Upfront payments, milestone payments and annual maintenance fees under license agreements are expensed in the period in which they are incurred.

**Patent Costs**— All patent-related costs incurred in connection with filing and prosecuting patent applications are expensed as incurred due to the uncertainty about the recovery of the expenditure. Amounts incurred are classified as general and administrative expenses.

**Debt Issuance Costs**— Debt issuance costs consist of payments made to secure commitments under certain debt financing arrangements. These amounts are recognized as interest expense over the period of the financing arrangement using the effective interest method. If the financing arrangement is canceled or forfeited, or if the utility of the arrangement to the Company is otherwise compromised, these costs are recognized as interest expense immediately. The Company's consolidated financial statements present debt issuance costs related to a recognized debt liability as a direct reduction from the carrying amount of that debt liability.

**Stock-Based Compensation**— The Company measures all stock-based awards granted to employees and directors based on the fair value on the date of the grant and recognizes compensation expense for those awards, net of estimated forfeitures, over the requisite service period, which is generally the vesting period of the respective award. The Company issues stock-based awards

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

with service-based vesting conditions and records the expense for these awards using the straight-line method. The Company has not issued any stock-based awards with performance-based vesting conditions.

Effective January 1, 2019, the Company adopted ASU No. 2018-7, *Compensation—Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting* (“ASU 2018-7”), which expands the scope of Topic 718 to include share-based payment awards to nonemployees. As a result, stock-based awards granted to non-employees are accounted for in the same manner as awards granted to employees and directors as described above. The impact of adoption of this new guidance did not have a material impact on the Company’s consolidated financial statements. Prior to the adoption of ASU 2018-7, for stock-based awards granted to non-employee consultants, compensation expense was recognized over the period during which services were rendered by such non-employee consultants until completed. At the end of each financial reporting period prior to completion of the service, the fair value of these awards was remeasured using the then-current fair value of the Company’s common stock and updated assumption inputs in the Black-Scholes option-pricing model.

The Company classifies stock-based compensation expense in its consolidated statement of operations in the same manner in which the award recipient’s payroll costs are classified or in which the award recipient’s service payments are classified.

The Company recognizes compensation expense for only the portion of awards that are expected to vest. In developing a forfeiture rate estimate, the Company has considered its historical experience to estimate pre-vesting forfeitures for service-based awards. The impact of a forfeiture rate adjustment is recognized in full in the period of adjustment, and if the actual forfeiture rate is materially different from the Company’s estimate, the Company may be required to record adjustments to stock-based compensation expense in future periods.

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model. Prior to March 13, 2019, the Company had been a private company and lacked company-specific historical and implied volatility information for its common stock. Therefore, the Company estimates its expected common stock price volatility based on the historical volatility of publicly traded peer companies and expects to continue to do so until it has adequate historical data regarding the volatility of its own traded stock price. The expected term of the Company’s stock options has been determined utilizing the “simplified” method for awards that qualify as “plain-vanilla” options. The expected term of stock options granted to non-employee consultants is equal to the contractual term of the option award. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield considers the fact that the Company has never paid cash dividends on common stock and does not expect to pay any cash dividends in the foreseeable future.

**Preferred Stock Warrant Liability**—Prior to the Merger with Arsanis, the Company classified warrants for the purchase of shares of its convertible preferred stock (see Note 11) as a liability on its consolidated balance sheets as these warrants are freestanding financial instruments that may have required the Company to transfer assets upon exercise. The warrant liability, which consisted of warrants for the purchase of Series A and Series B convertible preferred stock, was initially recorded at fair value upon the date of issuance of each warrant and was subsequently remeasured to fair value at each reporting date. Changes in the fair value of the warrant liability are recognized as a component of other income (expense), net in the consolidated statement of operations. Concurrent with the closing of the Merger, all X4 preferred stock was converted to common stock and the X4 preferred stock warrants converted to warrants for the purchase of Arsanis common stock. The Company assessed the features of the warrants and determined that they qualify for classification as permanent equity. Accordingly, the Company remeasured the warrants to fair value upon the closing of the Merger and reclassified the resulting warrant liability to additional paid-in capital (see Note 11).

**Derivative Liabilities: Genzyme Contingent Payment**—The Company’s license agreement with Genzyme Corporation (“Genzyme”) (see Note 5) contains a contingent payment obligation that required the Company to make a cash payment to Genzyme upon a change of control event of the Company. The contingent payment obligation met the definition of a derivative instrument as the contingent payment obligation was not clearly and closely related to its host instrument and was a cash-settled liability. Accordingly, the Company classified this derivative as a liability within other liabilities (non-current) on its condensed consolidated balance sheet. The derivative liability was initially recorded at fair value on the date of entering into the license agreement and was subsequently remeasured to fair value at each reporting date. Changes in the fair value of this derivative liability were recognized as a component of other income (expense), net in the condensed consolidated statement of operations. The Merger with Arsanis (see Note 1) 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. As a result, on March 13, 2019, the closing date of the Merger with Arsanis, the derivative liability was remeasured to fair value, which was \$0, and subsequent changes in fair value will be no.

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

longer be recognized in the consolidated statements of operations because the contingent payment obligation to Genzyme expired at that time.

**Derivative Liabilities: Hercules Loan Redemption Feature**—The Company's Hercules loan (see Note 8) contains a redemption feature that, upon an event of default, provides Hercules the option to accelerate and demand repayment of the debt, including a prepayment premium. The redemption feature meets the definition of a derivative instrument as the repayment of the debt contains a substantial premium, resulting in the redemption feature not being clearly and closely related to its host instrument. Accordingly, the Company classifies this derivative as a liability within other liabilities (non-current) on its condensed consolidated balance sheets. The derivative liability was initially recorded at fair value on the date of the Hercules Loan Agreement and is subsequently remeasured to fair value at each reporting date. Changes in the fair value of this derivative liability, which is included in other non-current liabilities, are recognized as a component of other income (expense), net in the condensed consolidated statement of operations. Changes in the fair value of this derivative liability will continue to be recognized until all amounts outstanding under the Hercules Loan Agreement are repaid or until the Hercules Loan Agreement is terminated. During the three and nine months ended September 30, 2019, there have been no changes to the fair value of this liability.

**Comprehensive Loss**— Comprehensive loss includes net loss as well as foreign currency translation adjustments. For the three and nine months ended September 30, 2019, comprehensive loss includes \$77 thousand and \$119 thousand of foreign currency translation adjustments, respectively.

**Income Taxes**— The Company accounts for income taxes using the asset and liability method, which requires the recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been recognized in the consolidated financial statements or in the Company's tax returns. Deferred tax assets and liabilities are determined based on the difference between the financial statement and tax basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Changes in deferred tax assets and liabilities are recorded in the provision for income taxes. The Company assesses the likelihood that its deferred tax assets will be recovered from future taxable income and, to the extent it believes, based upon the weight of available evidence, that it is more likely than not that all or a portion of the deferred tax assets will not be realized, a valuation allowance is established through a charge to income tax expense. Potential for recovery of deferred tax assets is evaluated by estimating the future taxable profits expected and considering prudent and feasible tax planning strategies.

The Company accounts for uncertainty in income taxes recognized in the consolidated financial statements by applying a two-step process to determine the amount of tax benefit to be recognized. First, the tax position must be evaluated to determine the likelihood that it will be sustained upon external examination by the taxing authorities. If the tax position is deemed more-likely-than-not to be sustained, the tax position is then assessed to determine the amount of benefit to recognize in the consolidated financial statements. The amount of the benefit that may be recognized is the largest amount that has a greater than 50% likelihood of being realized upon ultimate settlement. The provision for income taxes includes the effects of any resulting tax reserves, or unrecognized tax benefits, that are considered appropriate as well as the related net interest and penalties.

**Net Income (Loss) per Share**—For periods prior to the Merger with Arsanis on March 13, 2019, the Company follows the two-class method when computing net income (loss) per share as the Company had issued shares that met the definition of participating securities. The two-class method determines net income (loss) per share for each class of common and participating securities according to dividends declared or accumulated and participation rights in undistributed earnings. The two-class method requires income available to common stockholders for the period to be allocated between common and participating securities based upon their respective rights to receive dividends as if all income for the period had been distributed.

Basic net income (loss) per share attributable to common stockholders is computed by dividing the net income (loss) attributable to common stockholders by the weighted average number of shares of common stock outstanding for the period. Basic shares outstanding includes the weighted average effect of the Company's prefunded warrants issued in April 2019, the exercise of which requires little or no consideration for the delivery of shares of common stock. Diluted net income (loss) attributable to common stockholders is computed by adjusting net income (loss) attributable to common stockholders to reallocate undistributed earnings based on the potential impact of dilutive securities. Diluted net income (loss) per share attributable to common stockholders is computed by dividing the diluted net income (loss) attributable to common stockholders by the weighted average number of common shares outstanding for the period, including potential dilutive common shares. For

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

purpose of this calculation, outstanding stock options, convertible preferred stock and warrants to purchase shares of convertible preferred stock are considered potential dilutive common shares.

The Company's convertible preferred stock contractually entitled the holders of such shares to participate in dividends but did not contractually require the holders of such shares to participate in losses of the Company. Accordingly, in periods in which the Company reports a net loss attributable to common stockholders, such losses are not allocated to such participating securities. In periods in which the Company reports a net loss attributable to common stockholders, diluted net loss per share attributable to common stockholders is the same as basic net loss per share attributable to common stockholders, since dilutive common shares are not assumed to have been issued if their effect is anti-dilutive. The Company reported a net loss attributable to common stockholders for the three and nine months ended September 30, 2019 and 2018.

***Recently Adopted Accounting Pronouncements***

In February 2016, the FASB issued ASU No. 2016-2, *Leases (Topic 842)* ("ASU 2016-2" or "ASC 842"), which sets out the principles for the recognition, measurement, presentation and disclosure of leases for both parties to a contract (i.e., lessees and lessors). The new standard requires lessees to apply a dual approach, classifying leases as either finance or operating leases based on the principle of whether or not the lease is effectively a financed purchase by the lessee. This classification will determine whether lease expense is recognized based on an effective interest method or on a straight-line basis over the term of the lease. A lessee is also required to record a right-of-use asset and a lease liability for all leases with a term of greater than 12 months regardless of their classification. Leases with a term of 12 months or less may be accounted for similar to prior guidance for operating leases. The Company adopted the new lease standard as of the required effective date of January 1, 2019 using a modified retrospective transition approach applied to leases existing as of, or entered into after, January 1, 2019. The adoption had no impact on accumulated deficit. The new lease standard provides a number of optional practical expedients in transition. The Company applied the package of practical expedients to leases that commenced prior to the effective date, whereby it will elect to not reassess the following: (i) whether any expired or existing contracts contain leases; (ii) the lease classification for any expired or existing leases; and (iii) initial direct costs for any existing leases. The Company also elected the short-term lease recognition exemption for all leases that qualify, where a right-of-use asset or lease liability will not be recognized for short-term leases. Upon the adoption of ASC 842, the Company recorded \$2.5 million of operating lease liabilities and \$2.0 million of operating lease right-of-use assets on its consolidated balance. The adoption did not have a material impact on the Company's statement of operations, statement of comprehensive loss or statement of cash flows.

In January 2017, the FASB issued ASU No. 2017-4, *Intangibles-Goodwill and Other (Topic 350): Simplifying the Test for Goodwill Impairment*. The new standard simplifies the subsequent measurement of goodwill by eliminating the second step of the goodwill impairment test. This ASU will be applied prospectively and is effective for annual or interim goodwill impairment tests in fiscal years beginning after December 15, 2019 with early adoption permitted. The Company has adopted this guidance on January 1, 2019 and will apply it to its annual impairment test, and any interim impairment tests during the year ending December 31, 2019.

In April 2017, the FASB issued ASU 2017-8, *Receivables – Nonrefundable Fees and Other Costs* ("Subtopic 310-20"). The new standard amends the amortization period for certain purchased callable debt securities held at a premium by shortening the amortization period for the premium to the earliest call date. Subtopic 310-20 calls for a modified retrospective application under which a cumulative-effect adjustment will be made to retained earnings as of the beginning of the first reporting period in which the guidance is adopted. The Company adopted this guidance, effective January 1, 2019, and its adoption had no impact on the Company's financial position, results of operations or cash flows.

In July 2017, the FASB issued ASU No. 2017-11, *Earnings Per Share (Topic 260), Distinguishing Liabilities from Equity (Topic 480), Derivatives and Hedging (Topic 815) (Part I) Accounting for Certain Financial Instruments with Down Round Features (Part II) Replacement of the Indefinite Deferral for Mandatorily Redeemable Financial Instruments of Certain Nonpublic Entities and Certain Mandatorily Redeemable Noncontrolling Interests with a Scope Exception* ("ASU 2017-11"). Part I applies to entities that issue financial instruments such as warrants, convertible debt or convertible preferred stock that contain down-round features. Part II replaces the indefinite deferral for certain mandatorily redeemable noncontrolling interests and mandatorily redeemable financial instruments of nonpublic entities contained within ASC Topic 480 with a scope exception and does not impact the accounting for these mandatorily redeemable instruments. For public entities, ASU 2017-11 is effective for annual reporting periods beginning after December 15, 2018, including interim periods within those fiscal years. The Company adopted ASU 2017-11 as of the required effective date of January 1, 2019. The adoption of ASU 2017-11 had no impact on the Company's financial position, results of operations or cash flows.

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

In June 2018, the FASB issued ASU 2018-7, which superseded Subtopic 505-50, *Equity—Equity-Based Payments to Non-Employees* and amends ASC 718 to include share-based payments issued to non-employees for goods or services. Consequently, the accounting for share-based payments to non-employees and employees will be substantially aligned. The Company adopted ASU 2018-7 on January 1, 2019 and the adoption of this standard had no impact on the Company's financial position, results of operations or cash flows.

**Recently Issued Accounting Pronouncements**

In June 2016, the FASB issued ASU 2016-13, *Credit Losses (Topic 326)* ("ASU 2016-13"). ASU 2016-13 requires that financial assets measured at amortized cost, such as trade receivables, be presented net of expected credit losses, which may be estimated based on relevant information such as historical experience, current conditions, and future expectation for each pool of similar financial asset. The new guidance requires enhanced disclosures related to trade receivables and associated credit losses. The guidance is effective beginning January 1, 2020. The Company does not expect the adoption of this standard to have a material impact on its consolidated financial statements and related disclosures.

In August 2018, the FASB issued ASU No. 2018-13, *Fair Value Measurement (Topic 820): Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement* ("ASU 2018-13"), which removes, adds and modifies certain disclosure requirements for fair value measurements in Topic 820. The Company will no longer be required to disclose the amount of and reasons for transfers between Level 1 and Level 2 of the fair value hierarchy as well as the valuation processes of Level 3 fair value measurements. However, the Company will be required to additionally disclose the changes in unrealized gains and losses included in other comprehensive income for recurring Level 3 fair value measurements and the range and weighted average of assumptions used to develop significant unobservable inputs for Level 3 fair value measurements. ASU 2018-13 is effective for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2019. The amendments relating to additional disclosure requirements will be applied prospectively for only the most recent interim or annual period presented in the initial year of adoption. All other amendments will be applied retrospectively to all periods presented upon their effective date. The Company does not believe that the adoption of ASU 2018-13 will have a material impact on its consolidated financial statements.

In August 2018, the FASB issued ASU 2018-15, *Intangibles—Goodwill and Other—Internal-Use Software (Subtopic 350-40): Customer's Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That Is a Service Contract*, ("ASU 2018-15"). The amendments in this update align the requirements for capitalizing implementation costs incurred in a hosting arrangement that is a service contract with the requirements for capitalizing implementation costs incurred to develop or obtain internal-use software (and hosting arrangements that include an internal-use software license). The accounting for the service element of a hosting arrangement that is a service contract is not affected by the amendments in this update. The new standard will be effective beginning January 1, 2020 and early adoption is permitted. The amendments in this update should be applied either retrospectively or prospectively to all implementation costs incurred after the date of adoption. While the Company continues to evaluate the impact of the new guidance, the Company does not believe that the adoption of ASU 2018-15 will have a material impact on its consolidated financial statements.

In November 2018, the FASB issued ASU 2018-18, *Clarifying the Interaction between Topic 808 and Topic 606* ("ASU 2018-18"). ASU 2018-18 clarifies the interaction between the accounting guidance for collaborative arrangements and revenue from contracts with customers. The amendments become effective January 1, 2020. Early adoption, including adoption in any period, is permitted. The new guidance is required to be applied retrospectively as of the date of adoption of the new revenue guidance under ASC 606. The Company is evaluating the impact, if any, of this guidance on its consolidated financial statements and related disclosures and the timing of adoption.

In April 2019, the FASB issued ASU 2019-4 *Codification Improvements to Topic 326 Financial Instruments- Credit Losses, Topic 815, Derivatives and Hedging, and Topic 825* ("ASU 2019-4"). ASU 2019-4 clarifies the accounting treatment for the measurement of credit losses under ASC 236 and provides further clarification of ASU 2017-12, *Derivative and Hedging (Topic 815): Targeted Improvements to Accounting for Hedging Activities* and ASU 2016-1, *Financial Instruments—Overall (Subtopic 825-10): Recognition and Measurement of Financial Assets and Financial Liabilities*. ASU 2019-4 is effective for fiscal years beginning after December 15, 2019, including interim periods within those fiscal years. The Company does not anticipate that the adoption of ASU 2019-4 will have a material impact on its consolidated financial statements and related disclosures.

**3. Merger Accounting**

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

On March 13, 2019, the Company completed its merger with Arsanis. Based on the Exchange Ratio of 0.5702, immediately following the Merger, former Arsanis stockholders, Arsanis option holders and other persons holding securities or other rights directly or indirectly convertible, exercisable or exchangeable for Arsanis common stock owned approximately 31.3% of the outstanding capital stock of the combined organization on a fully diluted basis, and former X4 stockholders, holders of options or warrants to acquire X4 capital stock and other persons holding securities and other rights directly or indirectly convertible, exercisable or exchangeable for X4 capital stock owned approximately 68.7% of the outstanding capital stock of the combined organization on a fully diluted basis. At the closing of the Merger, all shares of X4 common stock and X4 preferred stock then outstanding were exchanged for Arsanis common stock.

In addition, pursuant to the terms of the Merger Agreement, the Company, for accounting purposes, assumed all outstanding stock options to purchase shares of Arsanis common stock at the closing of the Merger. At the closing of the Merger, such stock options became options to purchase an aggregate of 271,230 shares of the Company's common stock after giving effect to the Reverse Stock Split.

The total purchase price paid in the Merger has been allocated to the tangible and intangible assets acquired and liabilities assumed of Arsanis based on their fair values as of the completion of the Merger, with the excess allocated to goodwill. The following summarizes the preliminary estimate of the purchase price paid in the Merger (in thousands, except share and per share amounts):

Number of shares of the combined organization owned by Arsanis stockholders(1)	2,440,582
Multiplied by the fair value per share of Arsanis common stock(2)	\$ 18.66
Fair value of consideration issued in effect the Merger	\$ 45,541
Fair value of replacement awards held by former employees, board of directors and consultants of Arsanis that were vested as of the Merger.	\$ 817
Purchase price:	<u>\$ 46,358</u>

- (1) The number of shares of 2,440,582 represents the historical 14,643,737 shares of Arsanis common stock outstanding immediately prior to the closing of the Merger, adjusted for the Reverse Stock Split.
- (2) Based on the last reported sale price of Arsanis common stock on the Nasdaq Global Market on March 13, 2019, the closing date of the Merger, and gives effect to the Reverse Stock Split.

The following summarizes the allocation of the purchase price to the net tangible and intangible assets acquired (in thousands):

Cash, cash equivalents and restricted cash	\$ 26,406
Other current assets	2,147
Property and equipment, net	68
IPR&D indefinite-lived intangible assets	4,900
Other assets, non-current	486
Current liabilities	(5,205)
Loans payable	(8,713)
Other liabilities, non-current	(840)
Goodwill	27,109
Purchase price	<u>\$ 46,358</u>

The goodwill of \$27.1 million is not tax deductible and represents the excess of the consideration paid over the fair value of assets acquired and liabilities assumed. Goodwill is mainly attributable to the enhanced value of the combined company, as reflected in the increase in market value of the Arsanis common shares following the announcement of the Merger with X4. During the quarter ended June 30, 2019, goodwill was adjusted by \$298 thousand to reflect a change in the allocation of purchase price to the fair value of an acquired operating lease as of March 13, 2019. There have been no other changes in the value of goodwill from the acquisition date to September 30, 2019, and there were no indicators of impairment as of September 30, 2019.

The Company incurred costs directly related to the Merger of approximately \$1 million for the nine months ended September 30, 2019.

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

The in-process research and development intangible assets represent the acquisition-date fair value of three development programs acquired from Arsanis, which the Company refers to as the ASN200, ASN300 and ASN500 programs. The fair value of the IPR&D intangible assets was based on assumptions that market participants would use in pricing the assets, based on the most advantageous market for the assets assuming highest and best use. The fair value was determined by estimating the costs to develop the project into commercially viable products, estimating the resulting revenue from the projects, and discounting the projected net cash flows to present value using a weighted average discount rate of 20%. These IPR&D intangible assets are not amortized, but rather are reviewed for impairment on an annual basis or more frequently if indicators of impairment are present, until the project is completed, abandoned or transferred to a third party. As further described in Note 16, during the three months ended September 30, 2019, the Company entered into arrangements with two third parties that transferred the rights to develop and commercialize the programs underlying the IPR&D intangible assets. Accordingly, the Company derecognized the IPR&D intangible assets through a charge to "loss on transfer of non-financial assets" during the three months ended September 30, 2019.

The following supplemental unaudited pro forma information presents the Company's financial results as if the acquisition of Arsanis had occurred on January 1, 2018:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
(in thousands)	(unaudited)		(unaudited)	
Revenue	\$ —	\$ —	\$ —	\$ —
Net loss	\$ (17,714)	\$ (19,713)	\$ (46,876)	\$ (58,564)

The above unaudited pro forma information was determined based on the historical GAAP results of the Company and Arsanis. The unaudited pro forma consolidated results are not necessarily indicative of what the Company's consolidated results of operations would have been if the acquisition was completed on January 1, 2018. The unaudited pro forma consolidated net loss includes pro forma adjustments primarily relating to the reclassification of transaction costs and severance payments directly related to the closing of the Merger of \$2.7 million from the nine months ended September 30, 2019 to the nine months ended September 30, 2018. There were no material pro forma adjustments for the three month periods ended September 30, 2019 or September 30, 2018.

#### **4. License, Collaboration, and Funding Agreements**

##### ***Genzyme Agreement***

In July 2014, the Company entered into a license agreement (the "Genzyme Agreement") with Genzyme pursuant to which the Company was granted an exclusive license to certain patents and intellectual property owned or controlled by Genzyme related to the CXCR4 receptor to develop and commercialize products containing licensed compounds (including but not limited to X4P-1) for all therapeutic, prophylactic and diagnostic uses, with the exception of autologous and allogenic human stem cell therapy. Under the terms of the Genzyme Agreement, the Company is obligated to use commercially reasonable efforts to develop and commercialize licensed products for use in the field in the United States and at least one other major market country. The Company has the right to grant sublicenses of the licensed rights that cover X4P-001 to third parties.

In exchange for these rights, in August 2014, the Company made an upfront payment of \$50 thousand to Genzyme. The Company accounted for the acquisition of technology as an asset acquisition because it did not meet the definition of a business. The Company recorded the upfront payment as research and development expense in the consolidated statement of operations because the acquired technology represented in-process research and development and had no alternative future use. In August 2015, as a result of the closing of the Company's Series A preferred stock financing, the Company made an additional cash payment of \$300 thousand to Genzyme and issued to Genzyme 107,371 shares of its common stock, as adjusted for the 1-for-6 Reverse Stock Split and Exchange Ratio, each as required by the Genzyme Agreement. The \$300 thousand payment and the \$734 thousand fair value of the 107,371 shares of common stock issued to Genzyme were recorded as research and development expense in the consolidated statements of operations and comprehensive loss for the year ended December 31, 2015. Prior to the Merger with Arsanis, Genzyme had the right to require the Company to repurchase all, but not less than all, of these shares of common stock at any time during the term of the Genzyme Agreement for a price of \$0.01 per share. Due to this redemption feature, the shares of common stock issued to Genzyme were classified outside of stockholders' deficit on the consolidated balance sheets as of December 31, 2018. On March 13, 2019, the closing date of the Merger with Arsanis, these redeemable common shares were exchanged for common shares and, as a result, the fair value of the shares was reclassified to permanent equity.

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

Under the Genzyme Agreement, the Company is obligated to pay Genzyme milestone payments in the aggregate amount of up to \$25.0 million, contingent upon the achievement by the Company of certain late-stage regulatory and sales milestones with respect to licensed products. The Company is also obligated to pay Genzyme tiered royalties based on net sales of licensed products that the Company commercializes under the agreement. The 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 the Company is required to obtain a license from any third party to the extent the Company's 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 the Company enters into a sublicense under the Genzyme Agreement, the Company will be obligated to pay Genzyme a percentage of certain upfront fees, maintenance fees, milestone payments and royalty payments paid to the Company by the sublicensee.

Under the Genzyme Agreement, the Company will itself manufacture and supply, or enter into manufacturing or supply agreements with Genzyme or third parties to manufacture and supply, clinical and commercial supplies of licensed compounds and each licensed product. The Company is also responsible for all costs related to the filing, prosecution and maintenance of the licensed patent rights.

The Genzyme Agreement will remain in effect until the expiration of the royalty term in all countries for all licensed products. The Genzyme Agreement may be terminated by either party with at least 90 days' notice in the event of material breach by the other party that remains uncured for 90 days, by either party for insolvency or bankruptcy of the other party, immediately by Genzyme if the Company challenges the licensed patents, or immediately by the Company if a material safety issue arises.

During the nine months ended September 30, 2019, the Company did not incur any payment obligations to Genzyme under the Genzyme Agreement.

***Georgetown Agreement***

In December 2016, the Company entered into a license agreement (the "Georgetown Agreement") with Georgetown University ("Georgetown") pursuant to which the Company obtained an exclusive, worldwide license to make, have made, use, sell, offer for sale and import of products covered by patent rights co-owned by Georgetown. The rights licensed to the Company are for all therapeutic, prophylactic and diagnostic uses in all disease indications in humans and animals.

Under the terms of the Georgetown Agreement, the Company paid a one-time only, upfront fee of \$50 thousand and the Company may be required to make milestone payments of up to an aggregate of \$800 thousand related to commercial sales of a product. The Company recorded the upfront payment as research and development expense in the consolidated statement of operations because the acquired technology represented in-process research and development and had no alternative future use.

Under the Georgetown Agreement, the Company is solely responsible for all development and commercialization activities and costs in its respective territories. The Company is also responsible for all costs related to the filing, prosecution and maintenance of the licensed patent rights.

The term of the Georgetown Agreement will continue until the expiration of the last valid claim within the patent rights covering the product. Georgetown may terminate the agreement in the event (i) the Company fails to pay any amount and fails to cure such failure within 30 days after receipt of notice, (ii) the Company defaults in its obligation to obtain and maintain insurance and fails to remedy such breach within 45 days after receipt of notice, or (iii) the Company declares insolvency or bankruptcy. The Company may terminate the Georgetown Agreement at any time upon at least 60 days' written notice.

During the nine months ended September 30, 2019, the Company did not incur any payment obligations to Georgetown under the Georgetown Agreement and no milestone payments were made or due under the Georgetown Agreement.

***Beth Israel Deaconess Medical Center Agreement***

In December 2016, the Company entered into a license agreement (the "BIDMC Agreement") with Beth Israel Deaconess Medical Center ("BIDMC"), pursuant to which the Company obtained an exclusive, worldwide license to make, have made,

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

use, sell, offer for sale and import products covered by patent rights co-owned by BIDMC. The rights licensed to the Company are for all fields of use.

Under the terms of the BIDMC Agreement, the Company paid a one-time, upfront fee of \$20 thousand and the Company is responsible for all future patent prosecution costs. The Company recorded the upfront payment as research and development expense in the consolidated statement of operations because the acquired technology represented in-process research and development and had no alternative future use.

The term of the BIDMC Agreement will continue until the expiration of the last valid claim within the patent rights covering the licensed products. BIDMC may terminate the agreement in the event (i) the Company fails to pay any amount and fails to cure such failure within 15 days after receipt of notice, (ii) the Company is in material breach of any material provision of the BIDMC Agreement and fails to remedy such breach within 60 days after receipt of notice, or (iii) the Company declares insolvency or bankruptcy. The Company may terminate the BIDMC Agreement at any time upon at least 90 days' written notice.

The Company did not incur any payment obligations under the BIDMC Agreement during the nine months ended September 30, 2019.

***Research and Development Incentive Program***

The Company participates in a research and development incentive program provided by the Austrian government whereby the Company is entitled to reimbursement by the Austrian government for a percentage of qualifying research and development expenses incurred by the Company's subsidiary in Austria. Under the program, the reimbursement rate for qualifying research and development expenses incurred by the Company through its subsidiary in Austria is 14% for the current year.

The Company recognizes incentive income from Austrian research and development incentives when qualifying expenses have been incurred, there is reasonable assurance that the payment will be received, and the consideration can be reliably measured. Management has assessed the Company's research and development activities and expenditures to determine which activities and expenditures are likely to be eligible under the research and development incentive program described above. At each reporting date, management estimates the reimbursable incentive income available to the Company based on available information at the time.

As of September 30, 2019, the amount due under the program is \$1.7 million, which amounts were included in grant and incentive receivables in the consolidated balance sheet. During the three and nine months ended September 30, 2019, the Company recorded \$90 thousands and \$226 thousand of income related to the program within the condensed consolidated statement of operations as "other income (expense)".

***Janssen License and Option Agreement***

On December 12, 2018, Arsanis entered into a patent license and option agreement with Janssen Pharmaceuticals, Inc. ("Janssen"), (the "Janssen License and Option Agreement"). Pursuant to the Janssen License and Option Agreement, Arsanis granted to Janssen (i) a non-exclusive license to specified patents in Arsanis's portfolio related to the ASN200 program, and (ii) an option for Janssen to acquire these patents in the future if specified conditions are met. Janssen agreed to pay Arsanis \$3.5 million within 15 business days after the December 12, 2018 effective date of the Janssen License and Option Agreement, in addition to a future \$0.5 million payment in the event Janssen exercises its option to acquire the relevant patents. Arsanis received the \$3.5 million and recognized this amount as revenue. Such revenue is not reflected in the consolidated financial statements of the Company as it occurred prior to the Merger.

***Abbisko Agreement***

In July 2019, the Company entered into a license agreement (the "Abbisko Agreement") with Abbisko Therapeutics Co., Ltd. ("Abbisko"). Under the terms of the Abbisko Agreement, the Company granted Abbisko the exclusive right to develop, manufacture and commercialize mavorixafor in mainland China, Taiwan, Hong Kong and Macau (referred to in the Abbisko Agreement as the "Abbisko Territory"). The agreement provides Abbisko with the exclusive rights in the Abbisko Territory to develop and commercialize mavorixafor in combination with checkpoint inhibitors or other agents in oncology indications. Pancreatic cancer, ovarian cancer and triple negative breast cancer will be explored initially. The Company retains the full rest-of-world rights to develop and commercialize mavorixafor outside of Greater China for all indications and the ability to utilize and data generated pursuant to the Abbisko collaboration for rest-of-world development. In addition, Abbisko has the right of

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

first refusal if the Company determines to pursue and additional products in the Abbisko Territory. In accordance with the Abbisko Agreement, the Company has agreed to enter into separate agreements whereby the Company would provide Abbisko with a clinical supply and, if the product is commercialized in the Abbisko Territory, a commercial supply of the licensed compound.

Pursuant to the Abbisko Agreement, upon the closing of a qualified financing (as defined in the Abbisko Agreement), Abbisko will make a one-time, non-refundable, non-creditable financial milestone payment in the low single-digit millions to the Company. The Company is also eligible to receive potential development and regulatory milestone payments and potential commercial milestone payments based on annual net sales of licensed products. The Company does not expect to receive such milestone payments in the next 12 months. Upon commercialization of mavorixafor in the Abbisko Territory, the Company is eligible to receive a tiered royalty, with a percentage range in the low double-digits, on net sales of approved licensed products. Abbisko is obligated to use commercially reasonable efforts to develop and commercialize X4P-001 in the Abbisko Territory. Abbisko has responsibility for all activities and costs associated with the further development, manufacture and commercialization of X4P-001 in the Abbisko Licensed Territory.

The Company evaluated the Abbisko Agreement under ASC 606 and determined the Abbisko Agreement contained a single performance obligation related to the exclusive license to develop and commercialize X4P-001 and the transfer of know-how that was satisfied at the inception of the arrangement. The transaction price related to the transfer of the license and know-how was fully constrained and the Company ascribed no transaction price to the development, regulatory and commercial milestones under the "most-likely amount" method. As part of its evaluation, the Company considered multiple factors: (i) the ability of Abbisko to achieve a qualified financing (as defined in the Abbisko Agreement) is dependent on many factors outside of its control (ii) regulatory approvals are outside of the control of Abbisko, and (iii) certain development and regulatory milestones are contingent upon the success of future clinical trials, if any, which is out of the control of Abbisko. Any consideration related to the initial transfer of the license and know-how will be recognized when it is probable that Abbisko will achieve the related financial milestone. Any consideration related to development and regulatory milestones will be recognized when the corresponding milestones are achieved. Any consideration related to sales-based milestones (including royalties) will be recognized when the related sales occur as these amounts have been determined to relate predominantly to the license granted to Abbisko and therefore are recognized at the later of when the performance obligation is satisfied or the related sales occur.

The Company determined that the future sale of clinical and commercial supply are optional goods that will be subject to the customer's future purchasing decisions and do not represent performance obligations in the Abbisko Agreement. The Company concluded that the amount to be charged for the clinical supply will be reflective of market value and, therefore, the Abbisko Agreement does not have a discount on such supply that would be accounted for as material right at the outset of the contract. In arriving at these conclusions, the Company considered the complexity of the manufacturing process for the licensed compound and the potential ability for Abbisko to obtain the compound directly from other manufactures in the future. The Company expects that it will recognize revenue at a point in time when such clinical supply (and commercial supply, if applicable) is delivered to Abbisko in the future.

The Company will re-evaluate the transaction price, including its estimated variable consideration for milestones included in the transaction price and all constrained amounts, in each reporting period and as uncertain events are resolved or other changes in circumstances occur.

## **5. Fair Value of Financial Assets and Liabilities**

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

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

**Fair Value Measurements as of September 30, 2019 Using:**

(in thousands)	Level 1	Level 2	Level 3	Total
<b>Assets:</b>				
Cash equivalents—money market funds	\$ —	\$ 74,883	\$ —	\$ 74,883
	<u>\$ —</u>	<u>\$ 74,883</u>	<u>\$ —</u>	<u>\$ 74,883</u>
<b>Liabilities: None</b>				

**Fair Value Measurements as of December 31, 2018 Using:**

(in thousands)	Level 1	Level 2	Level 3	Total
<b>Assets:</b>				
Cash equivalents—money market fund	\$ —	\$ 8,134	\$ —	\$ 8,134
	<u>\$ —</u>	<u>\$ 8,134</u>	<u>\$ —</u>	<u>\$ 8,134</u>
<b>Liabilities:</b>				
Preferred stock warrant liability	\$ —	\$ —	\$ 4,947	\$ 4,947
Derivative liability	—	—	201	201
	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 5,148</u>	<u>\$ 5,148</u>

As of September 30, 2019 and December 31, 2018, there were no transfers between Level 1, Level 2 and Level 3.

The Company's cash equivalents consisted of a money market fund invested in U.S. Treasury securities. The money market fund was valued using inputs observable in active markets for similar securities, which represents a Level 2 measurement in the fair value hierarchy.

**Valuation of Preferred Stock Warrant Liabilities**—The preferred stock warrant liability in the table above consists of the fair values of (i) warrants to purchase shares of Series A convertible preferred stock that were issued in 2015 and shares of Series B convertible preferred stock that were issued in 2017 and 2018 in connection with the Company's Series A and Series B convertible preferred stock financings, respectively (see Note 11), (ii) warrants to purchase shares of Series A convertible preferred stock that were issued in 2016 in connection with the Company's entering into a loan and security agreement with Silicon Valley Bank (see Note 8) and (iii) warrants to purchase shares of Series B convertible preferred stock that were issued or were issuable in 2018 in connection with the Company's entering into the Hercules Loan Agreement (see Note 8). The liability associated with the warrants was recorded at fair value on the dates the warrants were issued and exercisable and was subsequently remeasured to fair value at each reporting date through December 31, 2018. Upon the closing of the Merger on March 13, 2019, all X4 preferred stock warrants were converted to warrants for Company's common stock and, as a result, the warrants were adjusted to fair value and reclassified to permanent equity. The aggregate fair value of the warrant liability was determined based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy.

The Company used various valuation methods, including the Monte Carlo method, the option-pricing method and the hybrid method (which is a combination of an option-pricing method and a probability-weighted expected return method), all of which incorporate assumptions and estimates, to value the preferred stock warrants. Estimates and assumptions impacting the fair value measurement include the fair value per share of the underlying shares of the Company's Series A and Series B convertible preferred stock, risk free interest rate, expected dividend yield, expected volatility of the price of the underlying preferred stock, and either the remaining contractual term of the warrants (except for warrants that would be automatically exercised upon an initial public offering, in which case the remaining estimated term to automatic exercise was used). The most significant assumption in the Monte Carlo method, the option-pricing method and the hybrid method impacting the fair value of the preferred stock warrants is the fair value of the Company's convertible preferred stock as of each remeasurement date. The Company determines the fair value per share of the underlying preferred stock by taking into consideration the most recent sales of its convertible preferred stock, results obtained from third-party valuations and additional factors that are deemed relevant. As of December 31, 2018, the fair value of the Series A convertible preferred stock was \$1.70 per share and the fair value of the Series B convertible preferred stock was \$1.86 per share. There were no warrants for the purchase of convertible preferred shares as of September 30, 2019 as all such warrants were converted to warrants for the purchase of common stock upon the Merger. The Company was a private company prior to the Merger and lacked company-specific historical and implied volatility information of its stock. Therefore, the Company had estimated its expected stock volatility based on the historical

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

volatility of publicly traded peer companies for a term equal to the estimated remaining term of the warrants. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve for time periods approximately equal to the estimated remaining term of the warrants. The Company estimated a 0% expected dividend yield as the Company has never paid or declared dividends and does not intend to do so in the foreseeable future.

**Valuation of Derivative Liability**— The fair value of the derivative liability recognized in connection with the Genzyme Agreement (see Note 5) was determined based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy. The fair value of this derivative liability is reported within other liabilities on the consolidated balance sheets. The fair value of this derivative liability was estimated by the Company at each reporting date based, in part, on the results of third-party valuations, which were prepared using the option-pricing method or the hybrid method, each of which considered as inputs the type, timing and probability of occurrence of a change of control event, the potential amount of the payment under potential exit scenarios, the fair value per share of the underlying common stock and the risk-adjusted discount rate. As of December 31, 2018, the fair value of this derivative liability was \$183 thousand. The Merger (see Note 1) qualified as a change of control event, as defined in the Genzyme Agreement, but results in no payment being due to Genzyme under the Genzyme Agreement. As a result, on March 13, 2019, the closing date of the Merger with Arsanis, this derivative liability was remeasured to fair value, which was zero, and subsequent changes in fair value will no longer be recognized in the consolidated statements of operations because the contingent payment obligation to Genzyme expired at that time.

The fair value of the derivative liability recognized in connection with the Hercules Loan Agreement (see Note 8) was determined based on significant inputs not observable in the market, which represents a Level 3 measurement within the fair value hierarchy. The fair value of this derivative liability is reported within other liabilities on the consolidated balance sheets. The fair value of this derivative liability was estimated by the Company at each reporting date based, in part, on the results of third-party valuations, which were prepared based on a discounted cash flow model that considered the timing and probability of occurrence of a redemption upon an event of default, the potential amount of prepayment upon an event of default and the risk-adjusted discount rate. As of September 30, 2019 and December 31, 2018, the fair value of this derivative liability was immaterial.

The following table provides a roll-forward of the aggregate fair values of the Company's warrant liability and derivative liability, for which fair values are determined using Level 3 inputs:

(in thousands)	Preferred Stock Warrant Liability	Derivative Liability
Balance as of December 31, 2018	\$ 4,947	\$ 201
Change in fair value	288	(183)
Conversion of convertible preferred stock warrant into common stock warrant in connection with Merger	(5,235)	
Balance as of September 30, 2019	\$ —	\$ 18

**6. Property and Equipment, Net**

Property and equipment, net consisted of the following:

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

(in thousands)	September 30, 2019	December 31, 2018
Leasehold improvements	\$ 336	\$ 299
Furniture and fixtures	183	53
Computer equipment	52	56
Software	33	9
Lab equipment	76	—
	680	417
Less: Accumulated depreciation and amortization	(332)	(176)
	<u>\$ 348</u>	<u>\$ 241</u>

Depreciation and amortization expense related to property and equipment was \$71 thousand and \$77 thousand for the nine months ended September 30, 2019 and 2018 respectively.

### 7. Accrued Expenses

Accrued expenses consisted of the following

(in thousands)	September 30, 2019	December 31, 2018
Accrued employee compensation and benefits	\$ 2,204	924
Accrued external research and development expenses	2,195	754
Accrued professional fees	853	1,324
Other	445	249
	<u>\$ 5,697</u>	<u>\$ 3,251</u>

### 8. Long-Term Debt

Long-term debt consisted of the following:

(in thousands)	September 30, 2019	December 31, 2018
Principal amount of long-term debt	\$ 20,000	\$ 10,000
Less: Current portion of long-term debt	—	(1,687)
Long-term debt, net of current portion	20,000	8,313
Debt discount, net of accretion	(317)	(226)
Cumulative accretion of final payment due at maturity	303	58
Long-term debt, including accretion, net of current portion	<u>\$ 19,986</u>	<u>\$ 8,145</u>

#### ***SVB Loan Agreement***

In October 2016, the Company entered into a loan and security agreement with Silicon Valley Bank (“SVB”), which the Company refers to as the SVB Loan Agreement, pursuant to which SVB made certain term loans available to the Company. The SVB Loan Agreement provided for a term loan of up to \$6.0 million, which was borrowed by the Company in September 2017. Borrowings under the SVB Loan Agreement bore interest at a variable rate equal to 5.5% plus the greater of (i) 3.5% or (ii) *The Wall Street Journal* prime rate. In October 2018, in connection with entering into the Hercules Loan Agreement, the Company terminated the SVB Loan Agreement and repaid all amounts due under the SVB Loan Agreement of \$4.7 million, including outstanding principal, prepayment premiums and accrued interest. As of October 19, 2018, the date of repayment of all borrowings under the SVB Loan Agreement, the interest rate applicable to borrowings under the SVB Loan Agreement was 10.75%.

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

***Hercules Loan Agreements***

***Hercules Loan Agreement and First Amendment***

In October 2018, the Company entered into the Hercules Loan Agreement. The Hercules Loan Agreement provided for aggregate borrowings of up to \$3.0 million, consisting of (i) a term loan of up to \$8.0 million, which was available upon entering into the agreement, (ii) subject to specified financing conditions, an additional term loan of up to \$2.0 million, available for borrowing from January 1, 2019 to March 31, 2019, and (iii) subject to specified financing conditions and the receipt of the second tranche term loan in the amount of \$2.0 million described above, an additional term loan of up to \$3.0 million, available for borrowing until March 31, 2019. In October 2018, the Company borrowed \$8.0 million under the Hercules Loan Agreement. In December 2018, the Company entered into the First Amendment to the Hercules Loan Agreement (the “First Amendment”), which amended the available borrowing dates of the second tranche from between January 1, 2019 and March 31, 2019 to between December 11, 2018 and December 14, 2018 and amended the term loan maturity date to November 1, 2021. In December 2018, the Company borrowed the additional \$2.0 million provided under the Hercules Loan Agreement, as amended by the First Amendment. In March 2019, the conditions necessary for borrowing the remaining \$3.0 million under the Hercules Loan Agreement, as amended by the First Amendment, were not met and the borrowing capacity expired at that time.

In connection with entering into the Hercules Loan Agreement in October 2018, the Company issued to Hercules warrants for the purchase of 210,638 shares of Series B convertible preferred stock at an exercise price of \$1.88 per share (which were subsequently converted to warrants for the purchase of 20,016 shares of common stock at an exercise price of \$19.78 per share following the Merger). These warrants were immediately exercisable and expire in October 2028. In addition, in connection with entering into the First Amendment in December 2018, the Company agreed to issue to Hercules warrants for the purchase of a specified number of shares of convertible preferred stock at an aggregate exercise price of \$99. On March 18, 2019, as a result of the closing of the Merger with Arsanis on March 13, 2019, the Company issued to Hercules warrants for the purchase of 5,000 shares of common stock of the combined organization at an exercise price of \$19.80 per share, each of which reflected the share Exchange Ratio of 1-for-0.5702 applied in the Merger as well as the Reverse Stock Split effected by the combined organization on March 13, 2019. On October 19, 2018 and December 11, 2018, the dates the Company entered into the Hercules Loan Agreement and the First Amendment, respectively, the Company recorded the aggregate initial fair value of the warrants of \$132 thousand as a preferred stock warrant liability, with a corresponding amount recorded as a debt discount on the Company’s consolidated balance sheet. As of March 13, 2019, and December 31, 2018, the fair value of the warrants were \$326 thousand and \$282 thousand, respectively. Upon the closing of the Merger, the warrants were converted to warrants for common stock and are no longer adjusted to fair value.

***2019 Amended and Restated Loan Agreement***

In June 2019, the Company refinanced the Hercules Loan Agreement, as amended, and entered into an Amended and Restated Loan and Security Agreement (the “2019 Loan Agreement”) with Hercules. The 2019 Loan Agreement provides for aggregate maximum borrowings of \$35.0 million, of which \$10.0 million was previously outstanding. The Company agreed to borrow \$20.0 million as of the closing date on June 27, 2019, including \$10.0 million in new borrowings and \$10.0 million rolled over from the previous agreement. An additional \$5.0 million is available for borrowing through December 15, 2020 and, subject to approval by Hercules, an additional term loan of \$10.0 million is available through June 15, 2022. Borrowings under the 2019 Loan Agreement bear interest at a variable rate equal to the greater of (i) 8.75% or (ii) 8.75% plus *The Wall Street Journal* prime rate minus 6.0%. In an event of default, as defined in the 2019 Loan Agreement, and until such event is no longer continuing, the interest rate applicable to borrowings under the 2019 Loan Agreement would be increased by 4.0%.

Borrowings under the 2019 Loan Agreement are repayable in monthly interest-only payments through January 1, 2022, and in equal monthly payments of principal and accrued interest from February 1, 2022 until the maturity date of the loan, which is July 1, 2023. At the Company’s option, the Company may prepay all, but not less than all, of the outstanding borrowings, subject to a prepayment premium of up to 2.0% of the principal amount outstanding as of the date of repayment. In addition, the 2019 Loan Agreement provides for payments by the Company to Hercules of (i) \$0.8 million payable upon the earlier of November 1, 2021 or the repayment in full of all obligations under the 2019 Loan Agreement, and (ii) 4.0% of the aggregate principal drawn under the 2019 Loan Agreement payable upon the earlier of maturity or the repayment in full of all obligations under such agreement.

Borrowings under the 2019 Loan Agreement are collateralized by substantially all of the Company’s personal property and other assets except for the Company’s intellectual property (but including rights to payment and proceeds from the sale,

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

licensing or disposition of the intellectual property). Under the 2019 Loan Agreement, the Company has agreed to affirmative and negative covenants to which it will remain subject until maturity or repayment of the loan in full. The covenants include (a) maintaining a minimum liquidity amount of the lesser of (i) 125% of the aggregate principal amount of outstanding borrowings under the June 2019 Loan Agreement and (ii) 100% of the Company and its consolidated subsidiaries' cash and cash equivalents (other than up to \$2.5 million which may be held by an excluded subsidiary, as defined) in an account in which Hercules has a first priority security interest, as well as (b) restrictions on the Company's ability to incur additional indebtedness, pay dividends, encumber its intellectual property, or engage in certain fundamental business transactions, such as mergers or acquisitions of other businesses. The 2019 Loan Agreement also contains a covenant that requires the Company to repay its loan with Österreichische Forschungsförderungsgesellschaft mbH ("FFG") on or prior to December 31, 2019. The FFG Loan Agreement was settled during the three months ended September 30, 2019 as discussed further below.

The Company's obligations under the 2019 Loan Agreement are subject to acceleration upon occurrence of specified events of default, including payment default, insolvency and a material adverse change in the Company's business, operations or financial or other conditions. The Company concluded that the redemption feature meets the definition of an embedded derivative instrument as the repayment of the debt contains a substantial premium, resulting in the redemption feature not being clearly and closely related to the host instrument. The Company recorded the issuance-date fair value of the derivative liability of \$18 thousand as a component of debt issuance costs.

In connection with entering into the Hercules Loan Agreement and the First Amendment, the Company had deferred \$180 thousand associated with upfront (1) fees associated with entering into the agreement, (2) the fair value of the warrants issued and (3) the fair value of an embedded derivative associated with the accelerated redemption feature. The \$180 thousand is classified as a debt discount, which is reflected as a reduction of the carrying value of long-term debt on the Company's consolidated balance sheet and is being amortized to interest expense over the term of the loan using the effective interest method. As a result of the June 2019 refinancing and entering into the 2019 Loan Agreement, the Company considered whether the previous debt was either extinguished or modified based on the difference in the cash flows of the previous and new debt. The Company determined that Hercules Loan Agreement, as amended by the First Amendment, was modified. Accordingly, the unamortized debt discount of the previous debt and newly incurred fees paid to the lender related to the 2019 Loan Agreement will be amortized to interest expense over the life of the new debt arrangement using the effective interest method.

The Company recognized aggregate interest expense under the Hercules Loan Agreement, as amended by the First Amendment, and the 2019 Loan Agreement of \$382 and \$0 during the three months ended September 30, 2019 and 2018, respectively, and \$739 thousand and \$0 during nine months ended September 30, 2019 and 2018, respectively. Interest expense includes \$101 thousand and \$215 thousand for the three and nine months ended September 30, 2019, respectively, related to the accretion of the debt discount and the final payment. As of September 30, 2019 the unamortized debt discount was \$104 thousand. The annual effective interest rate on the 2019 Loan Agreement as of June 30, 2019 is 11.0%. There were no principal payments due or paid under the Hercules Loan Agreement, as amended by the First Amendment, during the nine months ended September 30, 2019. Principal payments begin in January 2022.

***FFG Loan Agreements***

Between September 2011 and March 2017, Arsanis GmbH entered into a series of funding agreements with FFG that provided for loans and grants to fund qualifying research and development expenditures of X4 GmbH on a project-by-project basis, as approved by FFG. Amounts due under the FFG loans bear interest at rates ranging from 0.75% to 2.00% per annum. Giving effect to the Settlement Agreement (as defined below), the loans matured at various dates between March 31, 2019 and March 31, 2021. Interest on amounts due under the loans is payable semi-annually in arrears, with all principal and remaining accrued interest due upon maturity.

On March 8, 2019, Arsanis, Merger Sub, X4 and Arsanis GmbH entered into a Settlement Agreement with FFG (the "Settlement Agreement") in respect to allegations by FFG in February 2019 that Arsanis and Arsanis GmbH breached certain reporting, performance and other obligations in connection with grants and loans made by FFG to Arsanis GmbH between September 2011 and March 2017 to fund qualifying research and development expenditures. Pursuant to the terms of the Settlement Agreement, in exchange for FFG's waiver of all claims against Arsanis and Arsanis GmbH (except for its claims for repayment of the loans and regular interest but including its waiver of claims for repayment of grants and interest exceeding regular interest), subject to compliance by Arsanis and Arsanis GmbH with the terms of the Settlement Agreement, Arsanis GmbH agreed to repay the outstanding loan principal (plus regular interest accrued thereon) on an accelerated payment schedule of three years instead of five years, with the final accelerated installment due and payable on June 30, 2021. The

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

parties also agreed, among other things, that (i) the portion of such loans to be repaid in 2019 will be \$9 million on the first business day following March 31, 2019, and such amount was paid on April 1, 2019, and (ii) until all of the loans have been repaid and subject to other terms specified in the Settlement Agreement, commencing April 30, 2019, a minimum cash balance equal to 70% of the then-outstanding principal amount of the loans will be maintained at X4 Pharmaceuticals (Austria) GmbH in an account held with an Austrian bank.

In September 2019, the Company repaid all amounts due under the FFG loans and recognized a loss on extinguishment of debt of \$66 thousand, which is included in other income (expense), net, related to the unamortized loan discount as of the extinguishment date.

As of September 30, 2019, future principal payments and the final payment due under the Company's loan agreements were as follows (in thousands):

Year Ending December 31,	Total
2019	—
2020	\$ —
2021	—
2022	11,897
2023	8,103
Long-term debt	<u>\$ 20,000</u>

**9. Leases**

Effective January 1, 2019, the Company adopted ASC 842 using the modified retrospective approach through a cumulative-effect adjustment and utilizing the effective date as its date of initial application, with prior periods unchanged and presented in accordance with the previous lease accounting guidance. Upon adoption, the Company recorded right-of-use assets of \$2.0 million and lease liabilities of \$2.5 million, of which \$1.9 million was classified as non-current and \$613 thousand as current. The difference between the value of the right-of-use asset and the lease liabilities related to \$12 thousand of net deferred, accrued and prepaid rent that was reclassified against the right-of-use asset upon adoption of ASC 842 on January 1, 2019. The Company has lease agreements for its facilities in Cambridge, Massachusetts, which is the Company's global headquarters, Vienna, Austria, which is the Company's research and development center, and Waltham, Massachusetts, which is the former headquarters of Arsanis. The Company plans to sublease its Waltham, Massachusetts facility. There are no restrictions or financial covenants associated with any of the lease agreements.

**Cambridge Lease**— In August 2017, the Company entered into a non-cancellable operating lease agreement for office space of approximately thirteen thousand square feet in Cambridge, Massachusetts ("Cambridge Lease") which expires on July 31, 2022. The Cambridge Lease includes an annual rent escalation clause and the Company has the option to extend the lease for one period of five additional years. Base rent is approximately \$825 thousand annually and the monthly rent expense is being recognized on a straight-line basis over the term of the lease as the Company amortizes the associated operating lease right-of-use asset. In addition to the base rent, the Company is also responsible for its share of operating expenses, electricity and real estate taxes, in accordance with the terms of lease. These costs are not included in the determination of the leases' right-of-use operating assets or lease operating liabilities.

**Waltham Lease**— On March 13, 2019, as part of its Merger with Arsanis, the Company acquired a non-cancellable operating lease for approximately six thousand square feet of office space in Waltham, Massachusetts ("Waltham Lease"). The Waltham Lease, as amended, commenced on January 1, 2019, and expires approximately 5 years from the commencement date. The base rent is approximately \$62 thousand annually. In addition to the base rent, the Company is also responsible for its share of operating expenses, electricity and real estate taxes, which costs are not included in the determination of the leases' right-of-use assets or lease liabilities. The Company has ceased using the leased space and is actively seeking to obtain a subtenant. As a result, the Company has adjusted the value of the right-of-use asset to its estimated fair value, which represents management's best estimate of sublease income that could be obtained for the space, less costs to obtain a sublease. The right-of-use asset is being amortized to rent expense over the 5 year term of the lease.

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

**Vienna Austria Lease**— On March 13, 2019, as part of its Merger with Arsanis, the Company acquired an operating lease for approximately four hundred square meters of laboratory and office space in Vienna, Austria, (the “Vienna Austria Lease”) which commenced on March 1, 2019, as amended, for a term of 2 years. The lease is cancellable by the Company upon three months’ notice with no penalty. The annual base rent is approximately \$155 thousand. The Company has classified this lease as a short-term lease as it is not reasonably certain that the Company will not terminate the lease within one year and, accordingly, has not recorded a right-of-use asset. Accordingly, rent expense is recorded on a straight-line basis as incurred over the term of the lease.

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

The components of lease expense for the three and nine months ended September 30, 2019 were as follows (dollars in thousands):

<b>Lease Cost</b>	<b>For the Three Months Ended September 30, 2019</b>	<b>For the Nine Months Ended September 30, 2019</b>
Fixed operating lease cost	\$ 225	\$ 610
Short-term lease costs	38	84
<b>Total lease expense</b>	<b>\$ 263</b>	<b>\$ 694</b>
<b>Other information</b>		
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows from operating leases	\$ 268	\$ 737
Leased assets obtained in exchange for new operating lease liabilities <sup>(1)</sup>		\$ 484
Weighted-average remaining lease term—operating leases		3.3 years
Weighted-average discount rate—operating leases		9.0 %

(1) Acquired in the Merger

Maturities of lease liabilities due under these lease agreements as of September 30, 2019 are as follows (in thousands):

<b>Maturity of lease liabilities</b>	<b>Operating Leases</b>
2019 (remainder of 2019)	\$ 274
2020	1,090
2021	1,098
2022	754
2023	263
Total lease payments	3,479
Less: interest	(454)
Total operating lease liabilities as of September 30, 2019	<b>\$ 3,025</b>

The Company adopted ASU 2016-2 on January 1, 2019 as noted above, and as required, the following disclosure is provided for periods prior to adoption. Future annual minimum lease payments due under the Company’s operating leases as of December 31, 2018 were as follows (in thousands):

<b>Year Ending December 31,</b>	<b>Operating Leases</b>
2019	\$ 810
2020	823
2021	835
2022	492
<b>Total</b>	<b>\$ 2,960</b>

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

**10. Commitment and Contingencies**

**Sponsored Research Agreement Commitments**—In April 2017, the Company entered into a sponsored research agreement with a university pursuant to which the Company and the university are conducting a research program related to understanding the mechanisms of failed long-term adaptive immunity in WHIM patients. Under the terms of the agreement, the Company agreed to provide funding for the research program of up to \$499 thousand over a three-year period. The agreement will remain in effect for three years, unless earlier terminated. The Company may terminate the agreement at any time upon at least 60 days' prior written notice. For the three and nine months ended September 30, 2019, the Company incurred \$41 thousand and \$124 thousand, respectively, of research and development expenses related to its payment obligations to the university under the agreement. As of September 30, 2019, the Company had non-cancelable purchase commitments under this agreement totaling \$98 thousand, with \$42 thousand committed in 2019 and \$56 thousand committed in 2020.

In May 2019, the Company amended its agreement with a clinical research organization (“CRO”) pursuant to which the Company and the CRO are conducting a global Phase 3 clinical trial of mavoxixafor for the treatment of WHIM syndrome. The Company may terminate the agreement by providing 30 days' notice and if such termination occurred, the Company would incur early termination fees of up to \$0.6 million based on a percentage of committed resources of the CRO as of the termination.

**Indemnification Agreements**— In the ordinary course of business, the Company may provide indemnification of varying scope and terms to vendors, lessors, business partners and other parties with respect to certain matters including, but not limited to, losses arising out of breach of such agreements or from intellectual property infringement claims made by third parties. In addition, the Company has entered into indemnification agreements with members of its board of directors and its executive officers that will require the Company to, among other things, indemnify them against certain liabilities that may arise by reason of their status or service as directors or officers. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. To date, the Company has not incurred any material costs as a result of such indemnification obligations. The Company is not currently aware of any indemnification claims and has not accrued any liabilities related to such obligations in its consolidated financial statements as of September 30, 2019 or December 31, 2018.

**License Agreements**— In February 2017, Arsanis entered into an option and license agreement with Adimab to obtain rights to certain RSV antibodies, which are being used in the “ASN500” program. The Company exercised this option for an up-front payment of \$250 thousand. In July 2019, as further described in Note 16, the Company transferred the intellectual property related to the ASN500 program through an exclusive, worldwide license with a third party. The Company remains obligated to make payments to Adimab based on future clinical and regulatory milestones of up to approximately \$25 million, as well as royalty payments on a product-by-product and country-by-country basis of a mid-single-digit percentage based on net sales of products based on certain RSV antibodies during the applicable term for such product in that country. The third party is required to fund any future payments that may become due to Adimab.

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

**11. Preferred and Common Stock Warrants**

Prior to the Merger (see Note 1), the Company issued warrants for the purchase of its preferred stock and had classified these preferred stock warrants as a liability on its consolidated balance sheet as the warrants were deemed to be freestanding financial instruments that may have required the Company to transfer assets upon exercise. The liability associated with each of these warrants was initially recorded at fair value upon the issuance date of each warrant and was subsequently remeasured to fair value as a component of other income (expense), net in the consolidated statement of operations. Upon the closing of the Merger, pursuant to the Merger Agreement, all of the outstanding X4 preferred stock was converted to Arsanis common stock and the X4 preferred stock warrants converted to warrants for the purchase of Arsanis common stock. The Company assessed the features of the warrants and determined that they qualify for classification as permanent equity upon the closing of the

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

Merger. Accordingly, the Company remeasured the warrants to fair value upon the closing of the Merger, which was \$.2 million at March 13, 2019, with \$288 thousand of expense recorded during the three months ended March 31, 2019. Upon the closing of the Merger, the warrant liability was reclassified to additional paid-in capital.

In connection with its issuance of common stock in a public offering that closed on April 16, 2019, the Company issued 3,900,000 Class A warrants, which have an exercise price of \$13.20 per warrant and are convertible into shares of the Company's common stock. These warrants expire on April 15, 2024 and were immediately exercisable upon issuance. In addition, the Company issued 2,130,000 pre-funded warrants for proceeds of \$10.999 per share. Each of the pre-funded warrants is convertible into one common share, has a remaining exercise price of \$0.001 per share and was immediately exercisable upon issuance.

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

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

As of September 30, 2019, the Company's outstanding warrants to purchase shares of common stock consisted of the following:

Issuance Date	Number of Shares of Common Stock Issuable	Exercise Price	Classification	Expiration Date
August 14, 2015	81,228	\$ 21.78	Equity	August 14, 2020
August 21, 2015	69,603	\$ 21.78	Equity	August 21, 2020
October 25, 2016	5,155	\$ 19.78	Equity	October 24, 2026
November 1, 2017	130,609	\$ 19.78	Equity	October 31, 2020
November 17, 2017	8,442	\$ 19.78	Equity	November 16, 2020
December 4, 2017	5,661	\$ 19.78	Equity	December 3, 2020
December 28, 2017	6,925	\$ 19.78	Equity	December 27, 2020
December 28, 2017	115,916	\$ 19.78	Equity	December 28, 2027
September 12, 2018	25,275	\$ 19.78	Equity	September 12, 2021
September 12, 2018	20,220	\$ 19.78	Equity	September 12, 2028
October 19, 2018	20,016	\$ 19.78	Equity	October 19, 2028
March 13, 2019	5,000	\$ 19.80	Equity	March 12, 2029
April 16, 2019	3,866,154	\$ 13.20	Equity	April 15, 2024
April 16, 2019	2,130,000	\$ 11.00	Equity	n/a
	6,490,204			

As of December 31, 2018, the Company's outstanding warrants to purchase shares of preferred stock (which converted into warrants to purchase common stock upon close of the Merger) consisted of the following (not adjusted for the Reverse Stock Split or Exchange Ratio):

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

December 31, 2018

Warrant Name	Issuance Date	Number of Shares of Preferred Stock Issuable	Exercise Price	Exercisable for	Classification	Expiration Date
Series A warrants	August 14, 2015	854,785	\$ 2.07	Series A	Liability	August 14, 2020
Series A warrants	August 21, 2015	732,453	\$ 2.07	Series A	Liability	August 21, 2020
SVB warrants	October 25, 2016	54,256	\$ 1.88	Series A	Liability	October 24, 2026
Series B warrants	November 1, 2017	1,374,435	\$ 1.88	Series B	Liability	October 31, 2020
Series B warrants	November 17, 2017	88,845	\$ 1.88	Series B	Liability	November 16, 2020
Series B warrants	December 4, 2017	59,576	\$ 1.88	Series B	Liability	December 3, 2020
Series B warrants	December 28, 2017	72,875	\$ 1.88	Series B	Liability	December 27, 2020
Series B warrants	December 28, 2017	1,219,815	\$ 1.88	Series B	Liability	December 28, 2027
Series B warrants	September 12, 2018	265,957	\$ 1.88	Series B	Liability	September 12, 2021
Series B warrants	September 12, 2018	212,765	\$ 1.88	Series B	Liability	September 12, 2028
Series B warrants	October 19, 2018	210,638	\$ 1.88	Series B	Liability	October 19, 2028
		5,146,400				

**12. Common Stock, Redeemable Common Stock, and Convertible Preferred Stock (converted to Common Stock)**

**Common Stock**— As of September 30, 2019 and December 31, 2018, the Company’s Certificate of Incorporation, as amended and restated, authorized the Company to issue 33,333,333 shares and 11,070,776, shares, respectively, of \$0.001 par value common stock. The voting, dividend and liquidation rights of the holders of the Company’s common stock are subject to and qualified by the rights, powers and preferences of the holders of the preferred stock. Each share of common stock entitles the holder to one vote on all matters submitted to a vote of the Company’s stockholders. Common stockholders are entitled to receive dividends, as may be declared by the board of directors, if any. No cash dividends have been declared or paid to date.

On April 12, 2019, the Company entered into an underwriting agreement with Cowen and Company, LLC and Stifel, Nicolaus & Company, Incorporated, as representatives of the several underwriters named therein pursuant to which it sold 5,670,000 shares of common stock and, in lieu of common stock, prefunded warrants to purchase 2,130,000 shares of common stock, and accompanying Class A warrants to purchase 3,900,000 shares of its common stock. The common stock was issued at a price to the public of \$11.00 per share and accompanying Class A warrants and the prefunded warrants were issued at a price of \$10.999 per prefunded warrant and accompanying Class A warrants. The Class A warrants have an exercise price of \$13.20, will expire five years from the date of issuance, and are immediately exercisable with certain restrictions. The gross proceeds from the offering were \$85.8 million before deducting underwriting discounts and offering expenses. The offering closed on April 16, 2019.

The Company evaluated the Class A and prefunded warrants for liability or equity classification in accordance with the provisions of ASC 480 *Distinguishing Liabilities from Equity*, and ASC 815-40, *Derivatives and Hedging*, and determined that equity treatment was appropriate because neither the Class A warrants nor prefunded warrants meet the definition of a liability instrument. Neither the Class A warrants nor the prefunded warrants are mandatorily redeemable and they do not embody an obligation for the Company to repurchase them in circumstances that are outside of the Company’s control that could require the transfer of assets. The funds received in the equity offering related to these warrants has been classified as permanent equity as the warrants are freestanding financial instruments that are legally detachable and separately exercisable from the common stock with which they were issued, are immediately exercisable, do not embody an obligation for the Company to repurchase its shares and permit the holders to receive a fixed number of common stock upon exercise. Furthermore, the delivery of common stock to the warrant holder upon exercise of the warrant is within the Company’s control.

**Redeemable Common Stock**— Pursuant to the requirements of the July 2014 license agreement with Genzyme (see Note 4), in August 2015, the Company issued to Genzyme for no additional consideration 107,371, as adjusted for the Reverse Stock Split and Exchange Ratio, shares of common stock, which had an aggregate fair value of \$734 thousand on the date of issuance. Genzyme had the right to require the Company to repurchase all, but not less than all, of these shares of common stock at any time during the term of the license agreement for a price of \$0.01 per share. Because of this redemption feature, the shares of

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

common stock issued to Genzyme were classified outside of stockholders' deficit on the consolidated balance sheets. As a result of the Merger, these shares were exchanged for common stock.

**Convertible Preferred Stock (converted to Common Stock)**—The Company has issued Series Seed convertible preferred stock (the "Series Seed preferred stock"), Series A convertible preferred stock (the "Series A preferred stock") and Series B convertible preferred stock (the "Series B preferred stock"). As of September 30, 2019 and December 31, 2018, the Company's Certificate of Incorporation, as amended and restated, authorized the Company to issue a total of 10,000,000 shares and 59,413,523 shares, respectively, of preferred stock, with a par value of \$0.001 per share.

The holders of Preferred Stock have liquidation rights in the event of a deemed liquidation that, in certain situations, are not solely within the control of the Company. Therefore, the Preferred Stock is classified outside of stockholders' deficit on the consolidated balance sheet.

As of September 30, 2019, there was no preferred stock outstanding. As of December 31, 2018, the preferred stock consisted of the following:

	<b>December 31, 2018</b>				
	<b>Preferred Stock Designated</b>	<b>Preferred Stock Issued and Outstanding</b>	<b>Carrying Value</b>	<b>Liquidation Preference</b>	<b>Common Stock Issuable Upon Conversion (1)</b>
Series Seed preferred stock	2,313,523	1,516,136	\$ 1,310	\$ 1,444	143,630
Series A preferred stock	22,000,000	19,946,862	32,480	47,624	1,895,610
Series B preferred stock	25,100,000	18,616,569	30,885	34,999	1,769,190
	<u>49,413,523</u>	<u>40,079,567</u>	<u>\$ 64,675</u>	<u>\$ 84,067</u>	<u>3,808,430</u>

(1) Adjusted to reflect Reverse Stock Split and Exchange Ratio.

### 13. Stock-Based Compensation

**Summary of Plans**—Upon completion of the Merger with Arsanis on March 13, 2019, X4's 2015 Employee, Director and Consultant Equity Incentive Plan, as amended (the "2015 Plan"), Arsanis' 2017 Equity Incentive Plan (the "2017 Plan") and Arsanis' 2017 Employee Stock Purchase Plan (the "2017 ESPP") were assumed by the Company. In June 2019, the Company adopted the 2019 Inducement Equity Incentive Plan (the "2019 Plan"). These plans are administered by the Board of Directors or, at the discretion of the Board of Directors, by a committee of the Board of Directors. The exercise prices, vesting and other restrictions are determined at the discretion of the Board of Directors, or its committee if so delegated, except that the exercise price per share of stock options may not be less than 100% of the fair market value of the share of common stock on the date of grant and the term of the stock option may not be greater than ten years. Incentive stock options granted to employees and restricted stock awards granted to employees, officers, members of the board of directors, advisors, and consultants of the Company typically vest over four years. Non-statutory options granted to employees, officers, members of the board of directors, advisors, and consultants of the Company typically vest over three or four years. Shares that are expired, terminated, surrendered or canceled under the Plans without having been fully exercised will be available for future awards. In addition, shares of common stock that are tendered to the Company by a participant to exercise an award are added to the number of shares of common stock available for the grant of awards.

**2015 Employee, Director and Consultant Equity Incentive Plan**—In 2015, the Board of Directors and shareholders of X4 adopted the 2015 Plan, which provided for the Company to grant incentive stock options or nonqualified stock options, restricted stock awards and other stock-based awards to employees, directors and consultants of the Company. Each stock option outstanding under the 2015 Plan at the effective time of the Merger was automatically converted into a stock option exercisable for a number of shares of the Company's common stock calculated based on the Exchange Ratio and the exercise price per share of such outstanding stock option.

The total number of shares of common stock that may be issued under the 2015 Plan is 969,340 shares, adjusted for the Exchange Ratio and Reverse Stock Split. Shares that are expired, forfeited, canceled or otherwise terminated without having been fully exercised will be available for future grant under the 2015 Plan. As of September 30, 2019, approximately 73,000 shares were available for future issuance under the 2015 Plan. In addition, shares of common stock that are tendered to the Company by a participant to exercise an award are added to the number of shares of common stock available for future grants.

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

**2017 Equity Incentive Plan**—In 2017, the Board of Directors and shareholders of Arsanis adopted the 2017 Plan, which provided for the Company to grant incentive stock options, non-qualified options, stock appreciation rights, restricted stock awards, restricted stock units and other stock-based awards. Incentive stock options may be granted only to the Company’s employees, including officers and directors who are also employees. Awards other than incentive stock options may be granted to employees, officers, members of the board of directors, advisors and consultants of the Company. The number of shares of common stock reserved for issuance under this plan will automatically increase on January 1 of each year, through January 1, 2027, in an amount equal to the lowest of 170,915 shares of the Company’s common stock (as adjusted for the Reverse Stock Split), 4.0% of the number of shares of the Company’s common stock outstanding on January 1 of each year and an amount determined by the Company’s Board of Directors. As of September 30, 2019, approximately 20,000 shares were available for future issuance under the 2017 Plan.

**2017 Employee Stock Purchase Plan**—In 2017, the Board of Directors and shareholders of Arsanis adopted the 2017 ESPP. The 2017 ESPP provides participating employees with the opportunity to purchase shares of the Company’s common stock at defined purchase prices over nine-month offering periods. For the nine months ended September 30, 2019, no shares of common stock were issued under the 2017 ESPP.

**2019 Inducement Equity Incentive Plan**—On June 17, 2019, the Board of Directors approved the adoption of the 2019 Plan, which provides for the Company to grant nonqualified stock options, restricted stock awards and other stock-based awards to new employees of the Company. Awards issued from the 2019 Plan are intended to be material inducements to each employee’s acceptance of employment with the Company in accordance with Nasdaq Listing Rule 5635(c)(4). The total number of shares of common stock that may be issued under the 2019 Plan is 150,000 shares. Shares that are expired, forfeited, canceled or otherwise terminated without having been fully exercised will be available for future grant under the 2019 Plan. In addition, shares of common stock that are tendered to the Company by a participant to exercise an award are added to the number of shares of common stock available for future grants. As of September 30, 2019, approximately 55,000 shares were available for future issuance under the 2019 Plan.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Risk-free interest rate	1.6 %	2.7 %	2.0 %	2.7 %
Expected term (in years)	6.04	5.95	6.01	5.91
Expected volatility	84.5 %	86.7 %	88.0 %	86.7 %
Expected dividend yield	0 %	0 %	0 %	0 %

**Stock Options**

The following table summarizes the Company’s stock option activity for the nine months ended September 30, 2019:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Contractual Term (Years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2018	797,931	\$ 8.29	8.42	\$ 6,486
Assumed as part of Merger with Arsanis	271,230	62.60		
Granted	481,660	15.48		
Exercised	(25,627)	6.90		
Forfeited	(254,053)	35.92		
Outstanding as of September 30, 2019	1,271,141	\$ 17.11	8.45	\$ 2,760
Exercisable as of September 30, 2019	480,025	\$ 21.49	7.15	\$ 1,709
Vested and expected to vest as of September 30, 2019	1,092,790	\$ 17.18	8.35	\$ 2,595

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

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

**Restricted Stock Units**— During the nine months ended September 30, 2019, the Company granted 116,689 restricted stock units to employees. The restricted stock units vest 25% annually on the grant anniversary over four years and had a grant date fair value of \$1.7 million, which will be recognized as stock-based compensation expense, net of estimated forfeitures, over the vesting period.

**Stock-Based Compensation**— Effective January 1, 2019, the Company adopted ASU 2018-7 and no longer remeasures the fair value of equity awards granted to non-employees at each reporting period end (see Note 2).

As of September 30, 2019, total unrecognized compensation expense related to unvested stock options and restricted stock units was \$6.7 million, which is expected to be recognized over a weighted average period of 3.2 years.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Research and development expense	\$ 301	\$ 86	\$ 599	\$ 175
General and administrative expense	390	112	787	296
Total stock-based compensation	<u>\$ 691</u>	<u>\$ 198</u>	<u>\$ 1,386</u>	<u>\$ 471</u>

#### 14. Income Taxes

The Company did not record a federal or state income tax benefit for its losses for the three and nine months ended September 30, 2019 and 2018 due to the conclusion that a full valuation allowance is required against the Company's deferred tax assets.

#### 15. Net Loss per Share

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

(in thousands, except per share data)	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Numerator:				
Net loss	\$ (17,714)	\$ (8,602)	\$ (41,970)	\$ (22,003)
Accruing dividends on Series A convertible preferred stock	—	(756)	(592)	(2,244)
Adjustment to accumulated deficit in connection with repurchase of Series Seed convertible preferred stock	—	—	—	(22)
Net loss attributable to common stockholders	<u>\$ (17,714)</u>	<u>\$ (9,358)</u>	<u>\$ (42,562)</u>	<u>\$ (24,269)</u>
Denominator:				
Weighted average common shares outstanding—basic and diluted	14,562	459	9,866	459
Net loss per share attributable to common stockholders— basic and diluted	<u>\$ (1.22)</u>	<u>\$ (20.39)</u>	<u>\$ (4.31)</u>	<u>\$ (52.92)</u>

The Company has included 107,371 shares of redeemable common stock in its computation of basic and diluted weighted average common shares outstanding for the nine months ended September 30, 2019 and the three and nine months ended

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

September 30, 2018 as this class of stock participates in losses similarly to other common stockholders. Basic and diluted weighted average common shares outstanding for the three and nine months ended September 30, 2019 also includes the weighted average effect of 2,130,000 pre-funded warrants for the purchase of common shares, which were issued in April 2019 and for which the remaining unfunded exercise price is less than \$0.01 per share.

The Company's potentially dilutive securities included outstanding stock options, convertible preferred stock, and warrants to purchase shares of convertible preferred stock for the three and nine months ended September 30, 2018 and included outstanding stock options and warrants to purchase common stock for the three and nine months ended September 30, 2019. These potentially dilutive securities have been excluded from the computation of diluted net loss per share as the effect would be to reduce the net loss per share, and thus they are considered "anti-dilutive." Therefore, the weighted average number of common shares outstanding used to calculate both basic and diluted net loss per share attributable to common stockholders is the same. The Company excluded the following potential common shares, presented based on amounts outstanding at each period end and adjusted for the Exchange Ratio and Reverse Stock Split, from the computation of diluted net loss per share attributable to common stockholders for the periods indicated because including them would have had an anti-dilutive effect:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Options to purchase common stock	1,271,141	719,670	1,271,141	719,670
Convertible preferred stock (as converted to common stock)	—	3,808,894	—	3,808,894
Warrants to purchase common stock (excluding prefunded warrants, which are included in basic shares outstanding)	4,360,204	469,061	4,360,204	469,061
	<u>5,631,345</u>	<u>4,997,625</u>	<u>5,631,345</u>	<u>4,997,625</u>

**16. Loss on Transfer of Nonfinancial Assets**

During the quarter ended September 30, 2019, the Company entered into contractual arrangements with two third parties that transferred the rights to develop and commercialize the programs underlying IPR&D intangible assets acquired in the merger with Arsanis. As of September 30, 2019, all programs underlying IPR&D intangible assets acquired in the Merger were transferred to these third parties and the Company has no continuing involvement in any ongoing research and development activities associated with the programs. The Company concluded that these third parties are "non-customers" as the underlying development programs transferred to these third parties are focused on potential drug candidates that were not aligned with the Company's strategic focus and, therefore, are not an output of the Company's ordinary activities. Accordingly, the Company accounted for these transactions under ASC Topic 610-20, *Gains and Losses from the Derecognition of Nonfinancial Assets* ("ASC 610-20"). As a result of the transfer of control of the IPR&D projects to third parties, the Company derecognized the IPR&D intangible assets through a charge to "loss on transfer of non-financial assets" during the third quarter of 2019. The \$4.0 million loss includes the carrying value of the IPR&D of \$4.9 million, partially offset by \$0.9 million of non-refundable, upfront fees received from the third parties. In accordance with the contractual arrangements with these third parties, the Company is entitled to future fees that are contingent on the success of the third parties in advancing the IPR&D project toward commercialization. These future fees, if any, will be recognized in future periods as gains on the transfer of non-financial assets in accordance with ASC 610-20, which generally provides that the fees would be recognized when it is probable that a future reversal of the fees that would be material to cumulative fees recognized to date would not occur.

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

*You should read the following discussion and analysis of our financial condition and results of operations together with our consolidated financial statements and the related notes included elsewhere in this Quarterly Report on Form 10-Q. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report on Form 10-Q, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks, uncertainties and assumptions. Our actual results, performance or experience could differ materially from what is indicated by any forward-looking statement due to various important factors, risks and uncertainties, including, but not limited to, those set forth under "Cautionary Note Regarding Forward-Looking Statements" included elsewhere in this Quarterly Report on Form 10-Q or under "Risk Factors" in Item 1A of Part I of our Annual Report on Form 10-K for the fiscal year ended December 31, 2018, as updated by our Current Report on Form 8-K filed on April 11, 2019 and our subsequent filings under the Exchange Act.*

**Overview**

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

Our lead product candidate, mavorixafor (X4P-001), 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 a Phase 2 clinical trial in patients with Warts, Hypogammaglobulinemia, Infections, and Myelokathexis ("WHIM") syndrome, which is a rare, inherited primary immunodeficiency disease. On June 26, 2019, we announced the initiation of 4WHIM, a pivotal, 52-week Phase 3 global clinical trial of mavorixafor for the treatment of patients with WHIM syndrome. We plan to report top-line data from this trial in 2021. We are also investigating mavorixafor in combination with axitinib (Inlyta®) in the Phase 2a portion of an open-label Phase 1/2 clinical trial in clear cell renal cell carcinoma ("ccRCC"). Additionally, we have initiated a Phase 1b clinical trial of mavorixafor in another PI, severe congenital neutropenia ("SCN"), and we plan to initiate a Phase 1b clinical trial of mavorixafor in Waldenström macroglobulinemia ("WM") in the fourth quarter of 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 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 ("GBM"), and 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 Pipeline**

CANDIDATE	INDICATION	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3
Mavorixafor (X4P-001)	Warts, Hypogammaglobulinemia, Infections and Myelokathexis (WHIM) syndrome <sup>1</sup>	PHASE 3			
	Waldenström's Macroglobulinemia (WM)	PHASE 1B			
	Severe Congenital Neutropenia (SCN)	PHASE 2A			
X4P-002	Clear cell renal cell carcinoma <sup>2,3</sup> (ccRCC) (Combination with Inlyta®)	PHASE 2A			
X4P-003	Glioblastoma multiforme (GBM)	PHASE 1B			
	Primary immuno-deficiencies (PID)	PHASE 1B			

<sup>(1)</sup> Phase 2 open label extension study for WHIM ongoing and Phase 3 initiated.

<sup>(2)</sup> Two oncology trials have been concluded: P1b biomarker in melanoma and P1b in clear cell renal cell carcinoma ("ccRCC"). Positive data from ccRCC trial reported in September 2019 as noted in Recent Developments below.

<sup>(3)</sup> We intend to enter into a strategic partnership for future development and potential commercialization for mavorixafor for clear cell renal ccRCC and other potential solid tumor immuno-oncology indications.

**Recent Developments**

*Phase 2a Trial of Mavorixafor in Combination with Axitinib in Advanced Clear Cell Renal Cell Carcinoma Patients.*

On September 30, 2019, we announced positive results from the Phase 2a portion of our open-label Phase 1/2 clinical trial of mavorixafor in combination with axitinib (Inlyta®) in patients with advanced ccRCC. We expect to continue to explore the potential benefit of mavorixafor in underserved cancer patients with solid tumors, including as a potential triple combination agent in addition to tyrosine kinase inhibitor ("TKI") axitinib and checkpoint inhibitor therapies or in combination with other standard of care treatments.

*Received Orphan Drug Designation from European Commission for Mavorixafor for treatment of WHIM Syndrome*

On July 30, 2019, we announced that the European Commission, based on a favorable recommendation from the European Medicines Agency's Committee for Orphan Medical Products, has granted orphan drug designation to mavorixafor for the treatment of WHIM.

## X4 PHARMACEUTICALS, INC.

### Components of Results of Operations

#### Revenue

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

#### Operating Expenses

*Research and Development Expenses.* Research and development expenses consist primarily of costs incurred in connection with the discovery and development of our product candidates. We expense research and development costs as incurred. These expenses include:

- employee-related expenses, including salaries, related benefits and stock-based compensation expense, for employees engaged in research and development functions;
- expenses incurred in connection with the preclinical and clinical development of our product candidates, including under agreements with third parties, such as consultants and contract research organizations (“CROs”);
- the cost of manufacturing drug products for use in our preclinical studies and clinical trials, including under agreements with third parties, such as consultants and contract manufacturing organizations (“CMOs”);
- facilities, depreciation and other expenses, which include direct or allocated expenses for rent and maintenance of facilities and insurance;
- costs related to compliance with regulatory requirements; and
- payments made under third-party licensing agreements.

We recognize external development costs based on an evaluation of the progress to completion of specific tasks using information provided to us by our service providers. This process involves reviewing open contracts and purchase orders, communicating with our personnel to identify services that have been performed on our behalf, and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual costs. Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. Such amounts are recognized as an expense when the goods have been delivered or the services have been performed, or when it is no longer expected that the goods will be delivered or the services rendered. Upfront payments, milestone payments and annual maintenance fees under license agreements are expensed in the period in which they are incurred.

Our direct research and development expenses are tracked by product candidate and consist primarily of external costs, such as fees paid to outside consultants, CROs, CMOs and research laboratories in connection with our preclinical development, process development, manufacturing and clinical development activities. Our direct research and development expenses by product candidate also include fees incurred under third-party license agreements. We do not allocate employee costs and costs associated with our discovery efforts, laboratory supplies and facilities, including depreciation or other indirect costs, to specific product candidates because these costs are deployed across multiple programs and, as such, are not separately classified. We use internal resources primarily to conduct our research and discovery as well as for managing our preclinical development, process development, manufacturing and clinical development activities. These employees work across multiple programs and, therefore, we do not track our costs by product candidate.

The table below summarizes our research and development expenses incurred by product candidate:

(in thousands)	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Mavorixafor	\$ 5,137	\$ 3,124	\$ 13,407	\$ 8,410
X4P-002	78	—	219	—
X4P-003	276	—	494	—
Unallocated research and development expenses	3,098	3,034	8,978	7,247
Total research and development expenses	\$ 8,589	\$ 6,158	\$ 23,098	\$ 15,657

## X4 PHARMACEUTICALS, INC.

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. As a result, we expect that our research and development expenses will increase over the next several years on account of the Phase 3 pivotal clinical trial of mavorixafor for the treatment of patients with WHIM syndrome that we initiated in the second quarter of 2019, the Phase 1b clinical trial of mavorixafor in SCN that we recently initiated and a Phase 1b clinical trial of mavorixafor in WM that we plan to initiate in the fourth quarter of 2019. In addition, we expect research and development expenses to increase related to conducting preclinical development and pursuing initial clinical stages of our product candidates X4P-002 and X4P-003. While direct expenses related to our X4P-002 and X4P-003 product candidates have been relatively low as we paused the development of these candidates in favor of focusing our resources on the development of mavorixafor, we anticipate expenses related to our X4P-002 program will increase over the next several years as we resume work on X4P-002's preclinical development and initial clinical stage activities.

The successful development and commercialization of our product candidates is highly uncertain. At this time, we cannot reasonably estimate or know the nature, timing and costs of the efforts that will be necessary to complete the preclinical and clinical development of any of our product candidates. This is due to the numerous risks and uncertainties associated with product development and commercialization, including the following:

- the timing and progress of preclinical and clinical development activities;
- the number and scope of preclinical and clinical programs that we decide to pursue;
- our ability to maintain our current research and development programs and to establish new ones;
- establishing an appropriate safety profile with Investigational New Drug (“IND”)-enabling studies;
- successful patient enrollment in, and the initiation and completion of, clinical trials;
- the receipt of regulatory approvals from applicable regulatory authorities;
- the successful completion of clinical trials with safety, tolerability and efficacy profiles that are satisfactory to the U.S Food and Drug Administration (“FDA”) or any comparable foreign regulatory authority;
- the timing, receipt and terms of any marketing approvals from applicable regulatory authorities;
- our ability to establish new licensing or collaboration arrangements;
- establishing agreements with third-party manufacturers for clinical supply for our clinical trials and commercial manufacturing, if any of our product candidates is approved;
- development and timely delivery of clinical-grade and commercial-grade drug formulations that can be used in our clinical trials and for commercial launch;
- obtaining, maintaining, defending and enforcing patent claims and other intellectual property rights;
- launching commercial sales of our product candidates, if approved, whether alone or in collaboration with others; and
- maintaining a continued acceptable safety profile of the product candidates following approval.

A change in the outcome of any of these variables with respect to the development of our product candidates could significantly change the costs and timing associated with the development of that product candidate. We may never succeed in obtaining regulatory approval for any of our product candidates.

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

We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support our continued research activities and the clinical development of our product candidates. We also expect to continue to incur increased accounting, audit, legal, regulatory, compliance and director and officer insurance costs as well as investor and public relations expenses as a public company.

***Other Income (Expense), Net***

*Interest Income*

Interest income consists of interest earned on our cash equivalents, which consist of money market funds. Our interest income has not been significant due to low interest rates earned on invested balances.

*Interest Expense*

For the three and nine months ended September 30, 2019, interest expense primarily consists of interest on outstanding borrowings under our Hercules Loan Agreement and interest expense on loans with Österreichische Forschungsförderungsgesellschaft mbH ("FFG") acquired from Arsanis in March 2019. For the three and nine months ended September 30, 2018, interest expense consisted of interest on outstanding borrowing under our 2016 Silicon Valley Bank ("SVB") loan and security agreement (the "SVB Loan Agreement"). In October 2018, in connection with entering into the Hercules Loan Agreement, all amounts due under the SVB Loan Agreement were repaid and the SVB Loan Agreement was terminated. We expect that our interest expense will increase in 2019 as compared to 2018 due to the Hercules Loan Agreement, under which we borrowed an aggregate of \$10.0 million in the fourth quarter of 2018, and which we refinanced in June 2019 to borrow an additional \$10.0 million; and the FFG loan agreement acquired in our merger with Arsanis in March 2019, under which borrowings were approximately \$6.7 million during the period prior to being settled in September 2019.

Other Income (Expense), Net also includes of losses on the extinguishment of debt related to our loans from FFG, which we repaid and retired during the three months ended September 30, 2019.

*Change in Fair Value of Preferred Stock Warrant and Derivative Liabilities*

*Preferred Stock Warrants.* In connection with our preferred stock financings prior to our merger with Arsanis, we issued warrants to purchase shares of our preferred stock. Due to the liquidation requirements of these preferred shares, we classified these warrants as a liability on our consolidated balance sheet, which was remeasured to fair value at each reporting date with changes in the fair value reported as a component of other income (expense), net in our consolidated statements of operations and comprehensive loss. Upon the closing of the Merger on March 13, 2019, all outstanding preferred stock warrants become exercisable for Arsanis common stock; accordingly, the liability as of March 13, 2019 for these warrants was remeasured to fair value and reclassified to additional paid-in capital. As a result, following the closing of the Merger, we will no longer recognize changes in the fair value of these warrants as other income (expense), net in our consolidated statements of operations.

*Fair Value of Derivative Liability.* Our license agreement with Genzyme contains a contingent payment obligation that required us to make a cash payment to Genzyme upon a change of control event. The contingent payment obligation met the definition of a derivative instrument as the contingent payment obligation was not clearly and closely related to its host instrument and was a cash-settled liability. Accordingly, we classified this derivative as a liability within other liabilities (non-current) on our condensed consolidated balance sheet. The derivative liability was initially recorded at fair value on the date of entry into the license agreement and was subsequently remeasured to fair value at each reporting date. Changes in the fair value of this derivative liability were recognized as a component of other income (expense), net in the condensed consolidated statement of operations. The Merger with Arsanis 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. As a result, on March 13, 2019, the closing date of the Merger with Arsanis, the derivative liability was remeasured to fair value, which was zero, and subsequent changes in fair value will no longer be recognized in our consolidated statements of operations because the contingent payment obligation to Genzyme expired at that time.

***Income Taxes***

Since our inception, we have not recorded any income tax benefit for the net losses we have incurred in each year or for our earned research and development tax credits, as we believe, based upon the weight of available evidence, that it is more likely than not that all of our net operating loss carryforwards and tax credits will not be realized. Accordingly, we have recorded a full valuation allowance against our net deferred tax assets at each balance sheet date.

**X4 PHARMACEUTICALS, INC.**

**Results of Operations**

*Comparison of the Three and Nine Months Ended September 30, 2019 and 2018*

The following table summarizes the results of our operations for the periods indicated:

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2019	2018	Change	2019	2018	Change
	(in thousands)			(in thousands)		
<b>Operating expenses:</b>						
Research and development	\$ 8,589	\$ 6,158	\$ 2,431	\$ 23,098	\$ 15,657	\$ 7,441
General and administrative	4,383	2,387	1,996	13,726	5,374	8,352
Loss on transfer of non-financial assets	4,004	—	4,004	4,004	—	4,004
Total operating expenses	16,976	8,545	8,431	40,828	21,031	19,797
Loss from operations	(16,976)	(8,545)	(8,431)	(40,828)	(21,031)	(19,797)
<b>Other income (expense):</b>						
Interest income	464	51	413	927	187	740
Interest expense	(688)	(148)	(540)	(1,599)	(484)	(1,115)
Change in fair value of preferred stock warrant and derivative liabilities	—	40	(40)	(105)	(675)	570
Loss on extinguishment of debt	(566)	—	(566)	(566)	—	(566)
Other income (expense)	52	—	52	201	—	201
Total other income (expense), net	(738)	(57)	(681)	(1,142)	(972)	(170)
Net loss	\$ (17,714)	\$ (8,602)	\$ (9,112)	\$ (41,970)	\$ (22,003)	\$ (19,967)

**Research and Development Expenses**

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2019	2018	Change	2019	2018	Change
	(in thousands)			(in thousands)		
<b>Direct research and development expenses by product candidate:</b>						
Mavoxifafor (X4P-001)	\$ 5,137	\$ 3,124	\$ 2,013	\$ 13,407	\$ 8,410	\$ 4,997
X4P-002	78	—	78	219	—	219
X4P-003	276	—	276	494	—	494
Unallocated expense	3,098	3,034	64	8,978	7,247	1,731
Total research and development expenses	\$ 8,589	\$ 6,158	\$ 2,431	\$ 23,098	\$ 15,657	\$ 7,441

Research and development expenses were \$8.6 million for the three months ended September 30, 2019, compared to \$6.2 million for the three months ended September 30, 2018. Research and development expenses were \$23.1 million for the nine months ended September 30, 2019 compared to \$15.7 million for the nine months ended September 30, 2018. The increase in research and development expenses for the three and nine months ended September 30, 2019 was primarily due to an increase in direct expenses related to our mavoxifafor program and the costs incurred in advancing into the Phase 3 clinical development stage, and increased unallocated research and development costs, primarily associated with personnel and other costs associated with our research and development site in Vienna, Austria, acquired in the Merger. Research and development personnel-related costs for the three months ended September 30, 2019 and 2018 included stock-based compensation of \$0.3 million and \$0.1 million, respectively. Personnel-related costs, which are included in unallocated expenses, for the nine months ended September 30, 2019 and 2018 included stock-based compensation of \$0.6 million and \$0.2 million, respectively.

**General and Administrative Expenses** General and administrative expenses were \$4.4 million for the three months ended September 30, 2019, compared to \$2.4 million for the three months ended September 30, 2018. General and administrative expenses were \$13.7 million for the nine months ended September 30, 2019 compared to \$5.4 million for the nine months ended September 30, 2018. The increase in general and administrative expense for the three and nine months ended September

## X4 PHARMACEUTICALS, INC.

30, 2019 was primarily due to a significant increase in professional fees, including higher accounting and legal expenses, particularly related to the Merger. General and administrative expenses also increased due to higher legal costs incurred in connection with our intellectual property portfolio. In addition, general and administrative expenses increased for the three and nine months ended September 30, 2019 due to an increase in personnel-related costs due in an increase in head count to support the growth of our business and an increase in legal expenses as we transitioned to a public company. Personnel-related costs for the three months ended September 30, 2019 and 2018 included stock-based compensation of \$0.4 million and \$0.1 million, respectively. Personnel-related costs for the nine months ended September 30, 2019 and 2018 included stock-based compensation of \$0.8 million and \$0.3 million, respectively.

**Loss on Transfer of Nonfinancial Assets.** During the quarter ended September 30, 2019, we entered into contractual arrangements with two third parties that transferred the rights to develop and commercialize the programs underlying IPR&D intangible assets acquired in the merger with Arsanis. As a result of the transfer of control of the IPR&D projects to third parties, we recorded a charge of \$4.0 million to "loss on transfer of non-financial assets" during the third quarter of 2019. The loss included the carrying value of the IPR&D intangible assets prior to the transfer of control, partially offset by non-refundable, upfront fees received from the third parties. In accordance with the contractual arrangements with these third parties, we are entitled to future fees that are contingent on the success of the third parties in advancing the applicable IPR&D projects toward commercialization. There was no such loss in the three or nine months ended September 30, 2018.

### **Other Income (Expense), Net**

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2019	2018	Change	2019	2018	Change
	(in thousands)			(in thousands)		
Interest income	\$ 464	\$ 51	\$ 413	\$ 927	\$ 187	\$ 740
Interest expense	(688)	(148)	(540)	(1,599)	(484)	(1,115)
Change in fair value of preferred stock warrant and derivative liabilities	—	45	(45)	(288)	(264)	(24)
Change in fair value of derivative liability	—	(5)	5	183	(411)	594
Loss on extinguishment of debt	(566)	—	(566)	(566)	—	(566)
Other income (expense)	52	—	52	201	—	201
<b>Total other income (expense), net</b>	<b>\$ (738)</b>	<b>\$ (57)</b>	<b>\$ (681)</b>	<b>\$ (1,142)</b>	<b>\$ (972)</b>	<b>\$ (170)</b>

The increase in other expense of \$681 thousand during the three months ended September 30, 2019 as compared to the same period in the prior year was primarily due to a \$413 thousand increase in interest expense as a result of our increased borrowings during the three months ended September 30, 2019, primarily related to our Hercules and FFG loans, and increased losses related to the extinguishment of debt as a result of the retirement of our FFG loans at the end of September 30, 2019, partially offset by higher interest income derived from our money market funds. Other expenses for the nine months ended September 2019 also increased due to an increase in interest expense and increased losses related to the extinguishment of debt as a result of the retirement of our FFG loans at the end of the third quarter of 2019. These increases were partially offset by an increase in investment income derived from our money market funds.

### **Liquidity and Capital Resources**

#### *Source of Liquidity*

Since our inception, we have incurred significant operating losses and negative cash flows from our operations. We have not yet commercialized any product and we do not expect to generate revenue from sales of any products for several years, if at all. We expect that our research and development and general and administrative expenses will continue to increase and, as a result, we will need additional capital to fund our operations, which we may raise through a combination of equity offerings, debt financings, other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements.

We have funded our operations to date primarily with proceeds from sales of common stock, warrants and pre-funded warrants for the purchase of our preferred stock and our common stock, sales of preferred stock, proceeds from the issuance of convertible debt and borrowings under loan and security agreements. In March 2019, we completed a merger with Arsanis and

## X4 PHARMACEUTICALS, INC.

acquired \$26.4 million of cash, cash equivalents and restricted cash owned by Arsanis. In April 2019, we raised \$79.3 million, net of offering costs, through a public offering of common stock, pre-funded warrants to purchase shares of common stock, and accompanying Class A warrants to purchase shares of common stock. In June 2019, we received \$9.8 million in net proceeds as a result of refinancing our Hercules loan agreement and entering into the 2019 Loan Agreement as described further below.

As of September 30, 2019, we had cash and cash equivalents of \$76.3 million and we had restricted cash of \$0.7 million.

### **Cash Flows**

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

	Nine Months Ended September 30,	
	2019	2018
	(in millions)	
Net loss	\$ (42.0)	\$ (22.0)
Adjustments to reconcile net loss to net cash used in operating activities	7.1	1.3
Changes in operating assets and liabilities	(4.0)	2.0
Net cash used in operating activities	(38.8)	(18.7)
Net cash provided by investing activities	27.2	—
Net cash provided by financing activities	80.4	1.8
Impact of foreign exchange on cash and restricted cash	(0.3)	—
Net increase (decrease) in cash, cash equivalents and restricted cash	\$ 68.5	\$ (16.8)
Cash, cash equivalents and restricted cash, beginning of period	\$ 8.5	\$ 27.0
Cash, cash equivalents and restricted cash, end of period	\$ 77.0	\$ 10.2

**Operating Activities** During the nine months ended September 30, 2019, net cash used in operating activities was \$38.8 million, primarily resulting from our net losses of \$42.0 million, adjusted for noncash expenses of \$7.1 million and changes in our operating assets and liabilities of \$4.0 million. Noncash expenses primarily include the derecognition of IPR&D intangible assets included within "loss on transfer of non-financial assets" and stock-based compensation expense. Net cash used in changes in our operating assets and liabilities primarily consisted of a decrease in accounts payable and accrued expenses as a result of the timing of cash settlement of these liabilities. Net cash used in operating activities for the nine months ended September 30, 2018 was \$18.7 million, primarily reflecting our net losses of \$22.0 million, partially offset by noncash expenses of \$1.3 million and a change in operating assets and liabilities of \$2.0 million. The change in operating assets and liabilities was primarily due to an increase in accrued expenses due to higher professional fees and increases in research and development activities and costs, partially offset by a decrease in accrued employee compensation costs.

**Investing Activities** During the nine months ended September 30, 2019, net cash provided by investing activities consisted primarily of \$26.4 million of cash and restricted cash acquired in connection with our Merger. There was no comparable net cash provided by or used in investing activities during the same period in the prior year.

**Financing Activities** During the nine months ended September 30, 2019, net cash provided by financing activities was \$80.4 million, consisting primarily of \$79.3 million of net proceeds for the sale in April 2019 of our common stock and warrants and net proceeds of \$9.8 million received in our 2019 Loan Agreement with Hercules, partially offset by the settlement of our FFG loans. During the nine months ended September 30, 2018, net cash provided by financing activities was \$1.8 million, consisting primarily of net proceeds of \$4.5 million from the issuance of Series B convertible preferred stock, partially offset by \$1.1 million for the repurchase of Series Seed convertible preferred stock and principal repayments of \$1.5 million under our SVB Loan Agreement.

### **Loan and Security Agreements**

#### **Loan and Security Agreement with Silicon Valley Bank**

In October 2016, we entered into the SVB Loan Agreement, which provided for certain term loans, including a term loan of up to \$6.0 million, which we borrowed in June 2017. In October 2018, in connection with entering into the Hercules Loan Agreement (as described further below), we terminated the SVB Loan Agreement and repaid all amounts due under the SVB Loan Agreement, including unpaid principal of \$4.3 million, a final payment of \$0.3 million, a prepayment premium of \$87 thousand and accrued interest of \$23 thousand. As of October 19, 2018, the date of repayment of all borrowings under the SVB Loan Agreement, the interest rate applicable to borrowings under the SVB Loan Agreement was 10.75%. Our annual

## X4 PHARMACEUTICALS, INC.

effective interest rate of the SVB Loan Agreement was approximately 11.8% the period from January 1, 2018 through the date of repayment of all of the borrowings under the SVB Loan Agreement.

### *Loan and Security Agreement with Hercules Capital, Inc.*

In October 2018, we entered into a loan and Security Agreement (the "Hercules Loan Agreement") with Hercules Capital, Inc. ("Hercules"), which provided for aggregate borrowings of up to \$13.0 million, consisting of (i) a term loan of up to \$8.0 million, which was available upon entering into the Hercules Loan Agreement, (ii) subject to specified financing conditions, an additional term loan of up to \$2.0 million, available for borrowing from January 1, 2019 to March 31, 2019, and (iii) subject to specified financing conditions and the receipt of the second tranche \$2.0 million term loan described above, an additional term loan of up to \$3.0 million, available for borrowing until March 31, 2019. In October 2018, we borrowed \$8.0 million under the Hercules Loan Agreement.

In December 2018, we entered into the First Amendment to the Hercules Loan Agreement (the "First Amendment"), which amended the available borrowing dates of the second tranche from between January 1, 2019 and March 31, 2019 to between December 11, 2018 and December 14, 2018 and amended the term loan maturity date to November 1, 2021. In December 2018, we borrowed the additional \$2.0 million provided under the Hercules Loan Agreement, as amended. In March 2019, the conditions necessary for borrowing the remaining \$3.0 million under the Hercules Loan Agreement were not met and the borrowing capacity expired at that time.

In June 2019, we refinanced the Hercules Loan Agreement, as amended by the First Amendment, and entered into an Amended and Restated Loan and Security Agreement (the "2019 Loan Agreement"). The 2019 Loan Agreement provides for aggregate maximum borrowings of \$35.0 million of which \$10.0 million was previously outstanding. We agreed to borrow \$20.0 million as of the closing date on June 27, 2019, including \$10.0 million in new borrowings and \$10.0 million rolled over from the previous agreement. An additional \$5.0 million is available for borrowing through December 15, 2020 and, subject to approval by Hercules, an additional term loan of \$10.0 is available through June 15, 2022.

Borrowings under the 2019 Loan Agreement bear interest at a variable rate equal to the greater of (i) 8.75% or (ii) 8.75% plus *The Wall Street Journal* prime rate minus 6.0%. In an event of default, as defined in the 2019 Loan Agreement, and until such event is no longer continuing, the interest rate applicable to borrowings under the 2019 Loan Agreement would be increased by 4.0%. As of September 30, 2019, the interest rate applicable to borrowings under the 2019 Loan Agreement was 8.75%.

Borrowings under the 2019 Loan Agreement are repayable in monthly interest-only payments through January 1, 2022, and in equal monthly payments of principal and accrued interest from February 1, 2022 until the maturity date of the loan, which is July 1, 2023. At our option, we may prepay all, but not less than all, of the outstanding borrowings, subject to a prepayment premium of up to 2.0% of the principal amount outstanding as of the date of repayment. In addition, the 2019 Loan Agreement provides for payments of (i) \$0.8 million payable upon the earlier of November 1, 2021 or the repayment in full of all obligations under the June 2019 Loan Agreement, and (ii) 4.0% of the aggregate principal amount of advances drawn under the 2019 Loan Agreement payable upon the earlier of maturity or the repayment in full of all obligations under the agreement.

Borrowings under the 2019 Loan Agreement are collateralized by substantially all of our personal property and other assets except for our intellectual property (but including rights to payment and proceeds from the sale, licensing or disposition of the intellectual property). Under the 2019 Loan Agreement, we have agreed to affirmative and negative covenants to which we will remain subject until maturity or repayment of the loan in full. The covenants include (a) maintaining a minimum liquidity amount of the lesser of (i) 125% of the aggregate principal amount of outstanding borrowings under the 2019 Loan Agreement and (ii) 100% of our cash and cash equivalents (other than up to \$2.5 million which may be held by an excluded subsidiary, as defined) in an account in which Hercules has a first priority security interest, as well as (b) restrictions on our ability to incur additional indebtedness, pay dividends, encumber our intellectual property, or engage in certain fundamental business transactions, such as mergers or acquisitions of other businesses. The 2019 Loan Agreement also contains a covenant that requires us to repay our FFG loans on or prior to December 31, 2019. Our FFG loans were not modified as a result of this covenant. We were in compliance with all covenants under the June 2019 Loan Agreement as of June 30, 2019 and the FFG principal amounts are classified on the consolidated balance sheet in accordance with their contractual maturity dates.

Our obligations under the 2019 Loan Agreement are subject to acceleration upon occurrence of specified events of default, including payment default, insolvency and a material adverse change in our business, operations or financial or other conditions.

### **FFG Borrowings**

## X4 PHARMACEUTICALS, INC.

Between September 2011 and March 2017, Arsanis GmbH, a subsidiary of Arsanis, entered into a series of funding agreements with FFG that provided for loans and grants to fund qualifying research and development expenditures of Arsanis GmbH on a project-by-project basis, as approved by FFG. On March 8, 2019, Arsanis entered into a settlement agreement (“Settlement Agreement”), with FFG in respect of allegations that Arsanis breached certain reporting, performance and other obligations. Pursuant to the terms of the Settlement Agreement, in exchange for FFG’s waiver of all claims against Arsanis and Arsanis GmbH except for its claims for repayment of the loans and regular interest, including its waiver of claims for repayment of grants and interest exceeding regular interest, Arsanis GmbH agreed to repay the outstanding loan principal (plus regular interest accrued thereon) on an accelerated payment schedule of three years instead of the original five years, with the final accelerated installment due and payable on June 30, 2021. The Settlement Agreement also contained certain other restrictive covenants, including a requirement to maintain, as of April 30, 2019, a minimum cash balance equal to 70% of the outstanding principal amount of the loans in an account held with an Austrian bank, until all of the loans have been repaid and subject to other terms specified in the Settlement Agreement. Amounts due under the FFG loans bore interest at varying fixed rates ranging from 0.75% to 2.0% per annum.

During the quarter ended September 30, 2019, we re-paid the full amount due on the FFG loan of approximately \$6.5 million, and the FFG loans were retired. We recognized a loss on extinguishment of debt of \$0.7 million related to the unamortized debt discount as of the date of retirement.

### ***Funding Requirements***

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we advance the preclinical activities and clinical trials of our product candidates in development. In addition, we expect to continue to incur additional costs operating as a public company. Because of the numerous risks and uncertainties associated with research, development and commercialization of pharmaceutical product candidates, we are unable to estimate the exact amount of our working capital requirements. Our future funding requirements will depend on and could increase significantly as a result of many factors, including:

- the scope, number, initiation, progress, timing, costs, design, duration, any potential delays, and results of clinical trials and nonclinical studies for our current or future product candidates, particularly our Phase 3 pivotal clinical trial of mavoxixafor for the treatment of patients with WHIM syndrome, our Phase 1b clinical trial of mavoxixafor in SCN and our planned Phase 1b clinical trial of mavoxixafor in WM;
- the clinical development plans we establish for these product candidates;
- the number and characteristics of product candidates and programs that we develop or may in-license;
- the outcome, timing and cost of regulatory reviews, approvals or other actions to meet regulatory requirements established by the FDA and comparable foreign regulatory authorities, including the potential for the FDA or comparable foreign regulatory authorities to require that we perform more studies for our product candidates than those that we currently expect;
- our ability to obtain marketing approval for our product candidates;
- the cost of filing, prosecuting, defending and enforcing our patent claims and other intellectual property rights covering our product candidates, including any such patent claims and intellectual property rights that we have licensed from Genzyme pursuant to the terms of our license agreement with Genzyme;
- our ability to maintain, expand and defend the scope of our intellectual property portfolio, including the cost of defending intellectual property disputes, including patent infringement actions brought by third parties against us or our product candidates;
- the cost and timing of completion of commercial-scale outsourced manufacturing activities with respect to our product candidates;
- our ability to establish and maintain licensing, collaboration or similar arrangements on favorable terms and whether and to what extent we retain development or commercialization responsibilities under any new licensing, collaboration or similar arrangement;
- the cost of establishing sales, marketing and distribution capabilities for any product candidates for which we may receive regulatory approval in regions where we choose to commercialize our products on our own;
- the success of any other business, product or technology that we acquire or in which we invest;
- the costs of acquiring, licensing or investing in businesses, product candidates and technologies;

## X4 PHARMACEUTICALS, INC.

- our need and ability to hire additional management and scientific and medical personnel;
- the costs to continue to operate as a public company, including the need to implement additional financial and reporting systems and other internal systems and infrastructure for our business;
- market acceptance of our product candidates, to the extent any are approved for commercial sale; and
- the effect of competing technological and market developments.

We expect that our existing cash and cash equivalents will be sufficient to fund our operating expenses, capital expenditure requirements and debt service payments for at least the next 12 months. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our capital resources sooner than we expect.

Until such time, if ever, as we can generate substantial product revenue, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances, and marketing, distribution or licensing arrangements with third parties. In January 2019, Arsanis filed a universal shelf registration statement on Form S-3 with the SEC, which was declared effective in February 2019, pursuant to which Arsanis 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. Subsequently, in April 2019, we raised \$79.3 million, net of offering costs, for the sale of common stock, pre-funded warrants to purchase shares of common stock, and accompanying Class A warrants to purchase shares of our common stock. As of the date of this Quarterly Report, \$12 million of securities remain available for issuance under this shelf registration statement. In August 2019, we filed a shelf registration statement on Form S-3 with the SEC, which was declared effective in August 2019, 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. As of the date of this Quarterly Report, \$150 million of securities remain available for issuance under this shelf registration statement.

To the extent that we raise additional capital through future equity offerings or debt financings, the ownership interest of our stockholders may be materially diluted, and the terms of such securities could include liquidation or other preferences that adversely affect the rights of our stockholders. Debt financing and preferred equity financing, if available, may involve agreements that include restrictive covenants that limit our ability to take specified actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings or other arrangements when needed, we may be required to delay, reduce or eliminate our product development efforts or future commercialization efforts, or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

### **Critical Accounting Policies and Significant Judgments and Estimates**

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

While our significant accounting policies are described in more detail in Note 2 to our consolidated financial statements included elsewhere in this Quarterly Report, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our consolidated financial statements.

***Accrued Research and Development Expenses.*** As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued research and development expenses. This process involves reviewing open contracts and purchase orders, communicating with our applicable personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual costs. The majority of our service providers invoice us in arrears for services performed, on a pre-determined schedule or when contractual milestones are met; however, some require advance payments. We make estimates of our accrued expenses as of each balance sheet date in the consolidated financial statements based on facts and circumstances

## X4 PHARMACEUTICALS, INC.

known to them at that time. We periodically confirm the accuracy of these estimates with the service providers and make adjustments, if necessary. Examples of estimated accrued research and development expenses include fees paid to:

- vendors in connection with preclinical development activities;
- CROs and investigative sites in connection with preclinical studies and clinical trials; and
- CMOs in connection with the production of preclinical and clinical trial materials.

We base the expense recorded related to external research and development on our estimates of the services received and efforts expended pursuant to quotes and contracts with multiple CMOs and CROs that supply, conduct and manage preclinical studies and clinical trials on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the expense. Payments under some of these contracts depend on factors such as the successful enrollment of patients and the completion of clinical trial milestones. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from the estimate, we adjust the accrual or the amount of prepaid expenses accordingly.

Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in reporting amounts that are too high or too low in any particular period. To date, there have not been any material adjustments to our prior estimates of accrued research and development expenses.

**Stock-Based Compensation.** We measure all stock-based awards granted to employees, directors and consultants based on the grant-date fair value of the award and recognized compensation expense, net of estimated forfeitures, over the requisite service period, which is generally the vesting period of the respective award. The stock-based awards that we have issued to date include a service-based vesting condition and the expense for these awards is recognized using the straight-line method. To date, we have not issued stock-based awards with performance-based vesting conditions.

The fair value of stock option grants is estimated on the date of grant using the Black-Scholes option-pricing model, which uses as inputs the fair value of our common stock and assumptions we make for the volatility of our common stock, the expected term of the stock options, the risk-free interest rate for a period that approximates the expected term of our stock options and their expected dividend yield. Prior to the closing of the Merger and the listing of our common stock on the Nasdaq Capital Market, our board of directors historically determined, as of the date of each option grant and with input from our management, the assistance of a third-party valuation specialist the estimated fair value of our common stock on the date of grant based on a number of objective and subjective factors. Since the Merger and the listing of our common stock on the Nasdaq Capital Market, we have relied on the market price of our common stock to determine the fair value on the date of grant. As our common stock does not have a sufficient history of trading, we estimate our volatility based on the historical volatility of publicly traded peer companies. We estimate the expected term of our stock awards by utilizing the “simplified” method, which calculates the expected term based on weighted average midpoint of the award’s vesting and expiration dates. We determine the risk-free interest rate by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. We estimate that no dividends will be paid as we do not expect to pay cash dividends in the foreseeable future.

The assumptions underlying these valuations represent the best estimates of our management, which involve inherent uncertainties and the application of our judgment. As a result, if factors or expected outcomes change and we use significantly different assumptions or estimates, the resulting share-based compensation expense could be materially different.

**Valuation of Preferred Stock Warrant Liability.** In connection with our preferred stock financings prior to our merger with Arsanis in March 2019, we issued warrants to purchase shares of our preferred stock. We classified these warrants as liabilities on our consolidated balance sheet because they were deemed to be freestanding financial instruments that may have required us to transfer assets upon exercise. The warrant liability was initially recorded at fair value upon the date of issuance of each warrant and is subsequently remeasured to fair value at each reporting date as a component of other income (expense), net in our consolidated statements of operations. Upon the closing of the Merger on March 13, 2019, all outstanding preferred stock warrants become exercisable for Arsanis common stock and, accordingly, the warrant liability as of March 13, 2019 was remeasured to fair value and reclassified to additional paid-in capital. As a result, following the closing of the Merger, we no longer recognize changes in the fair value of the warrant liability as other income (expense), net in our consolidated statement of operations.

## X4 PHARMACEUTICALS, INC.

To value the preferred stock warrants, we used various valuation methods, including the Monte Carlo method, the option-pricing method and the hybrid method, all of which incorporated assumptions and estimates. We assessed these assumptions and estimates on a quarterly basis as additional information impacting the assumptions is obtained. Estimates and assumptions impacting the fair value measurement included the fair value per share of the underlying shares of our Series A and Series B preferred stock, risk-free interest rate, expected dividend yield, expected volatility of the price of the underlying preferred stock, and the remaining contractual term of the warrants (except for the warrants that would be automatically exercised upon an initial public offering, in which case the remaining estimated term to automatic exercise was used). The most significant assumption in the Monte Carlo method, the option-pricing method and the hybrid method impacting the fair value of the preferred stock warrants was the fair value of our preferred stock as of each remeasurement date. We determined the fair value per share of the underlying preferred stock by taking into consideration the most recent sales of our preferred stock, results obtained from third-party valuations and additional factors that are deemed relevant.

### **Emerging Growth Company Status**

We are an “emerging growth company,” or EGC, as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. We will remain an EGC until the earlier of (1) December 31, 2022 (2) the last day of the fiscal year in which we have total annual gross revenue of at least \$1.07 billion, (3) the last day of the fiscal year in which we are deemed to be a “large accelerated filer” as defined in Rule 12b-2 under the Exchange Act, which would occur if the market value of our common stock held by non-affiliates exceeded \$700.0 million as of the last business day of the second fiscal quarter of such fiscal year or (4) the date on which we have issued more than \$1.0 billion in non-convertible debt securities during the prior three-year period. The JOBS Act permits an EGC to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have irrevocably elected to “opt out” of this provision and, as a result, we will comply with new or revised accounting standards when they are required to be adopted by public companies that are not EGCs.

### **Off-Balance Sheet Arrangements**

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

### **Recently Issued Accounting Pronouncements**

A description of recently issued accounting pronouncements that may potentially impact our financial position and results of operations is disclosed in Note 2 to our consolidated financial statements included elsewhere in this Quarterly Report.

## **Item 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK**

As a smaller reporting company, as defined by Rule 12b-2 of the Exchange Act and in Item 10(f)(1) of Regulation S-K, we are electing scaled disclosure reporting obligations and therefore are not required to provide the information requested by this Item.

## **Item 4 CONTROLS AND PROCEDURES**

### **Evaluation of Disclosure Controls and Procedures**

Our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) as of the end of the period covered by this Form 10-Q, and have concluded that, based on such evaluation, our disclosure controls and procedures were effective as of September 30, 2019 at the reasonable assurance level to ensure that information required to be disclosed by us in the reports that we file or submit under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC’s rules and forms, and is accumulated and communicated to our management, including our principal executive officer and principal financial officers, as appropriate, to allow timely decisions regarding required disclosure.

### **Changes in Internal Control Over Financial Reporting**

There were no changes in our internal control over financial reporting that occurred during the three months ended September 30, 2019 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

X4 PHARMACEUTICALS, INC.

PART II: OTHER INFORMATION

**Item 1. LEGAL PROCEEDINGS**

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

**Item 1A. RISK FACTORS**

There have been no material changes to the risk factors described in our Annual Report on Form 10-K for the year ended December 31, 2018 filed with the SEC on March 11, 2019, as updated by the risk factors described in our Current Report on Form 8-K, filed with the SEC on April 11, 2019.

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

None.

**Item 3. DEFAULTS UPON SENIOR SECURITIES**

None.

**Item 4. MINE SAFETY DISCLOSURES**

Not applicable.

**Item 5. OTHER INFORMATION**

None.

**Item 6. EXHIBITS**

Exhibit No.	Exhibit Description	Filed Herewith	Incorporated by Reference herein from Form or Schedule	Filing Date	SEC File/Reg. Number
2.1	<a href="#">Second Amendment to Agreement and Plan of Merger, dated March 8, 2019, by and among the Company, Artemis AC Corp. and X4 Therapeutics, Inc. (formerly X4 Pharmaceuticals Inc.).</a>		8-K (Exhibit 2.1)	3/8/2019	001-38295
3.1	<a href="#">Restated Certificate of Incorporation of the Company</a>		8-K (Exhibit 3.1)	11/20/2017	001-38295
3.2	<a href="#">Certificate of Amendment (Reverse Stock Split) to the Restated Certificate of Incorporation of the Company</a>		8-K (Exhibit 3.1)	3/13/2019	001-38295
3.3	<a href="#">Certificate of Amendment (Name Change) to the Restated Certificate of Incorporation of the Company (Name Change) to the Restated Certificate of Incorporation of the Company</a>		8-K (Exhibit 3.2)	3/13/2019	001-38295
31.1	<a href="#">Certification of Principal Executive Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.</a>	X			

X4 PHARMACEUTICALS, INC.

31.2	<a href="#">Certification of Principal Financial Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.</a>	X
32.1*	<a href="#">Certification of Principal Executive Officer and Principal Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.</a>	X
101	Condensed Consolidated Balance Sheets (unaudited) as of September 30, 2019 and December 31, 2018, (ii) Condensed Consolidated Statements of Operations (unaudited) for the Three and Nine months ended September 30, 2019 and 2018, (iii) Condensed Consolidated Statements of Comprehensive Loss (unaudited) for the Three and Nine Months Ended September 30, 2019 and 2018, (iv) Condensed Consolidated Statements of Convertible Preferred Stock, Redeemable Common Stock and Stockholders' Equity (Deficit) (unaudited) for the Three and Nine Months Ended September 30, 2019 and 2018, and (v) Condensed Consolidated Statements of Cash Flows (unaudited) for the Nine Months Ended September 30, 2019 and 2018.	
101.INS+	XBRL Instance Document	
101.SCH+	XBRL Taxonomy Extension Schema Document	
101.CAL+	SXRL Taxonomy Extension Calculation Linkbase Document	
101.DEF+	XBRL Taxonomy Extension Definition Linkbase Document	
101.LAB+	XBRL Taxonomy Extension Label Linkbase Document	
101.PRE+	XBRL Taxonomy Extension Presentation Linkbase Document	

\* The certification attached as Exhibit 32.1 accompanying this Quarterly Report on Form 10-Q is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of the Company under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Quarterly Report on Form 10-Q, irrespective of any general incorporation language contained in such filing.

+ Submitted electronically herewith

**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 thereunto duly authorized.

**X4 PHARMACEUTICALS, INC.**

Date: November 7, 2019

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

Paula Ragan, Ph.D.

President, Chief Executive Officer and Secretary

Date: November 7, 2019

By: /s/ Adam S. Mostafa

Adam S. Mostafa

Chief Financial Officer and Treasurer

## CERTIFICATION

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

1. I have reviewed this Quarterly Report on Form 10-Q of X4 Pharmaceuticals, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 7, 2019

/s/ Paula Ragan, Ph.D.

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Paula Ragan, Ph.D.

President, Chief Executive Officer and Secretary  
(Principal Executive Officer)

## CERTIFICATIONS

I, Adam S. Mostafa, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of X4 Pharmaceuticals, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 7, 2019

/s/ Adam S. Mostafa

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

## CERTIFICATIONS UNDER SECTION 906

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

1. The Company's Quarterly Report on Form 10-Q for the period ended September 30, 2019, to which this Certification is attached as Exhibit 32.1 (the "Periodic Report") fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, and
2. The information contained in the Periodic Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

**IN WITNESS WHEREOF**, the undersigned have set their hands hereto as of the 7th day of November, 2019.

Dated: November 7, 2019

/s/ Paula Ragan, Ph.D.

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

Dated: November 7, 2019

/s/ Adam S. Mostafa

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

This certification accompanies the Form 10-Q to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of X4 Pharmaceuticals, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Form 10-Q), irrespective of any general incorporation language contained in such filing.