



## X4 Pharmaceuticals Reports Third Quarter 2019 Financial Results and Recent Business Highlights

November 7, 2019

*Received orphan drug designation from the European Commission for mavorixafor for treatment of WHIM Syndrome*

*Reported positive data from Phase 2a clinical trial of mavorixafor in combination with axitinib in advanced clear cell renal cell carcinoma patients*

*Hosts investment community conference call at 8:00 a.m. ET on November 7, 2019*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 7, 2019-- [X4 Pharmaceuticals, Inc.](https://www.x4pharma.com) (Nasdaq:XFOR), a clinical-stage biopharmaceutical company focused on the development of novel therapeutics for the treatment of rare diseases, today reported financial results for the third quarter ended September 30, 2019 and provided an update on recent developments in its business.

"Our core focus remains to advance our clinical programs with mavorixafor across our three lead rare disease indications. We are proud to report that we now have orphan drug designation in Europe, in addition to in the United States, for mavorixafor for treatment of Warts, Hypogammaglobulinemia, Infections, and Myelokathexis (WHIM) Syndrome. We recently commenced enrollment in our pivotal Phase 3 clinical trial for the treatment of WHIM syndrome and are activating clinical sites globally. We have also now initiated the Phase 1b clinical trial in Severe Congenital Neutropenia. Preparations are underway for the initiation of the Phase 1b clinical trial in Waldenström's macroglobulinemia later this quarter," stated Paula Ragan, Ph.D., President and Chief Executive Officer of X4.

### Clinical Development Update

In November 2019, X4 initiated a Phase 1b clinical trial of mavorixafor (X4P-001) for the treatment of Severe Congenital Neutropenia (SCN), a group of rare blood disorders characterized by abnormally low levels of neutrophils.

X4 commenced U.S. patient enrollment and is activating additional sites globally in the pivotal Phase 3 global clinical trial of mavorixafor in WHIM syndrome, a rare, inherited, primary immunodeficiency disease caused by genetic mutations in the CXCR4 receptor gene. The 52-week trial is designed to enroll 18 to 28 subjects in approximately 20 countries, followed by an open-label extension trial.

In September 2019, at the European Society for Medical Oncology (ESMO), X4 released positive results from the Phase 2a portion of its open-label Phase 1/2 clinical trial of mavorixafor in combination with axitinib (Inlyta®) in patients with advanced clear cell renal cell carcinoma (ccRCC). The combination therapy was observed to generally be well-tolerated with a manageable safety profile and demonstrated clinical improvement with encouraging median progression free survival (mPFS) in a heavily pretreated advanced ccRCC patient population. Results suggest that mavorixafor may enhance clinical response to axitinib and other tyrosine kinase inhibitors (TKIs) that target tumor angiogenesis, as well as immunotherapy agents. The Company is in ongoing dialogue with potential partners to explore the potential benefit of mavorixafor in underserved cancer patients with solid tumors, including as a potential triple combination agent in addition to TKI and checkpoint inhibitor therapies or in combination with other standard of care treatments.

### Business Update

In July 2019, X4 entered into an agreement with Abbisko Therapeutics where Abbisko agreed to develop and commercialize mavorixafor in combination with checkpoint inhibitors or other agents in Greater China for oncology indications with a focus on solid tumor indications. X4 retained full rest-of-world rights to develop and commercialize mavorixafor outside of Greater China for all indications, and the ability to utilize any data generated from the collaboration for X4's rest-of-world development programs.

In July 2019, X4 was notified that the European Commission (EC) had approved orphan drug designation (ODD) for mavorixafor for the treatment of WHIM syndrome, which follows the orphan drug designation X4 received from the U.S. Food and Drug Administration in October 2018 for the same indication.

In September 2019, X4 appointed William "Bill" E. Aliski to its Board of Directors. Mr. Aliski has more than two decades of biopharmaceutical executive leadership experience at both public and private companies, with significant expertise in global rare disease commercialization, including a particular focus on commercial strategy, pricing, reimbursement and market access.

In September 2019, X4 appointed Renato Skerlj, Ph.D., as its Senior Vice President, Research and Development. Dr. Skerlj has twenty-five years of experience leading the discovery and development of small molecule drugs to treat rare diseases, cancer, infection and neurodegenerative diseases. In addition, he is one of the original founders of X4 Pharmaceuticals.

In November 2019, X4 appointed Derek Meisner, J.D., as its General Counsel. Mr. Meisner brings more than two decades of experience providing counsel to public and private biotechnology companies across key legal and operational functions, including global regulatory compliance, financings, mergers and acquisitions, strategic partnerships and corporate governance.

### Third Quarter 2019 Financial Highlights

**Cash Position:** As of September 30, 2019, cash, cash equivalents and restricted cash were \$77.0 million, as compared to \$95.6 million as of June 30, 2019. This decrease reflected cash used to fund operating activities during the third quarter and included a \$6.5 million cash payment for the settlement of X4's loans with Österreichische Forschungsförderungsgesellschaft mbH (FFG). This repayment was part of the debt refinancing executed with Hercules in June 2019, under which X4 has a remaining \$5 million in borrowing availability.

**Research and Development Expenses (R&D):** R&D expenses were \$8.6 million for the third quarter of 2019, as compared to \$8.9 million for the second quarter of 2019.

**General and Administrative Expenses (G&A):** G&A expenses were \$4.4 million for the third quarter of 2019, as compared to \$4.6 million for the second quarter of 2019.

**Loss on Transfer of Non-Financial Assets:** During the third quarter of 2019, X4 transferred to third parties the rights to develop and commercialize the programs underlying its in-process research and development (IPR&D) intangible assets, which were obtained in its merger with Arsanis. Accordingly, X4 recorded a \$4.0 million loss during the three months ended September 30, 2019 reflecting the derecognition of the IPR&D intangible assets, partially offset by cash proceeds received in the quarter.

**Net Loss:** Net loss was \$17.7 million for the third quarter of 2019, or a net loss per basic and diluted share of \$1.22, as compared to a net loss of \$13.4 million for the second quarter of 2019, or a net loss per basic and diluted share of \$1.02. Excluding the loss on transfer of non-financial assets, net loss for the third quarter of 2019 was approximately the sum of the R&D and G&A expenses in the quarter.

X4 expects current cash and cash equivalents to be sufficient to fund operations into the first half of 2021.

### Conference Call and Webcast Information

X4 will host a conference call and webcast on November 7, 2019 at 8:00 a.m. ET to discuss these financial results and business highlights. The conference call can be accessed by dialing (866) 721-7655 from the United States or (409) 216-0009 internationally, followed by the conference ID: 4081686. The live webcast can be accessed on the investor relations section of X4's website at <http://investors.x4pharma.com/events-and-presentations>. Following the completion of the call, a webcast replay of the conference call will be available on X4's website for thirty days.

### About Mavorixafor

X4 Pharmaceuticals' lead product candidate, mavorixafor (X4P-001), is a potential first-in-class, once-daily, oral inhibitor of CXCR4, 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. Mavorixafor has demonstrated proof-of-concept in WHIM syndrome in a Phase 2 clinical trial, including clinically meaningful increases in neutrophil and lymphocyte biomarker counts, as well as a trend of reduction in infection rates and wart burden, and a favorable safety profile. Mavorixafor was designated orphan drug status by the U.S. Food and Drug Administration in 2018 and by the European Commission in 2019 for the treatment of WHIM syndrome, and is also in development for Severe Congenital Neutropenia (SCN), Waldenström's macroglobulinemia (WM), and clear cell renal cell carcinoma (ccRCC).

### About X4 Pharmaceuticals

X4 Pharmaceuticals is developing novel therapeutics designed to improve immune cell trafficking to treat rare diseases, including primary immunodeficiencies and certain cancers. The company's oral small molecule drug candidates antagonize the CXCR4 pathway, which plays a central role in immune surveillance. X4's most advanced product candidate, mavorixafor (X4P-001), is in a global Phase 3 pivotal trial in patients with WHIM syndrome, a rare, inherited, primary immunodeficiency disease, and is currently also under investigation in combination with axitinib in an open-label Phase 1/2 clinical trial in clear cell renal cell carcinoma (ccRCC), with several patients remaining on therapy over 12 months beyond the primary endpoint. X4 is further investigating mavorixafor in a Phase 1b clinical trial for the treatment of Severe Congenital Neutropenia (SCN). X4 is also planning to commence a clinical trial of mavorixafor with ibrutinib for the treatment of Waldenström's macroglobulinemia (WM) in 2019. X4 was founded and is led by a team with extensive biopharmaceutical product development and commercialization expertise and is committed to advancing the development of innovative medicines on behalf of patients with limited treatment options. X4 is a global company that is headquartered in Cambridge, Massachusetts with research offices based in Vienna, Austria. For more information, please visit [www.x4pharma.com](http://www.x4pharma.com).

### Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. The words "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Forward-looking statements include, but are not limited to, statements regarding X4's plans for the development of mavorixafor (X4P-001) or any of X4's other product candidates or programs, including the clinical trials X4 plans to initiate; the design, rate of patient enrollment and clinical site initiation for its clinical trials; the potential benefits of mavorixafor, including as a treatment for advanced renal cell carcinoma, WHIM, Waldenström's macroglobulinemia, or Severe Congenital Neutropenia; the safety, durability or efficacy of mavorixafor; X4's plans to announce future trial results; X4's ability to fund its future operations; and X4's plans to partner with current and future third-parties. These statements are subject to various risks and uncertainties, actual results could differ materially from those projected and X4 cautions investors not to place undue reliance on the forward-looking statements in this press release. These risks and uncertainties include, without limitation, the risk that trials and studies may be delayed and may not have satisfactory outcomes, potential adverse effects arising from the testing or use of mavorixafor or other product candidates, the risk that prior results, such as signals of safety, activity or durability of effect, observed from preclinical studies or clinical trials will not be replicated or will not continue in ongoing or future studies or trials involving X4's product candidates, the risk that X4 will be unable to identify collaboration partners or realize benefits from any existing or future collaborations, and the risk that costs required to develop mavorixafor or other product candidates or to expand X4's operations will be higher than anticipated. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, the risks and uncertainties described in the section entitled "Risk Factors" in X4's most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC), as updated by X4's Current Report on Form 8-K filed with the SEC on April 11, 2019, and in other filings X4 makes with the SEC from time to time. X4 undertakes no obligation to update the information contained in this press release to reflect subsequently occurring events or circumstances.

**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
Operating expenses:				
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	4,004	—	4,004	—
Total operating expenses	16,976	8,545	40,828	21,031
Loss from operations	(16,976 )	(8,545 )	(40,828 )	(21,031 )
Other income (expense), net	(738 )	(57 )	(1,142 )	(972 )
Net loss	(17,714 )	(8,602 )	(41,970 )	(22,003 )
Adjustments related to convertible preferred stock	—	(756 )	(592 )	(2,266 )
Net loss attributable to common stockholders	\$ (17,714 )	\$ (9,358 )	\$ (42,562 )	\$ (24,269 )
Net loss per share attributable to common stockholders—basic and diluted	\$ (1.22 )	\$ (20.39 )	\$ (4.31 )	\$ (52.92 )
Weighted average common shares outstanding—basic and diluted	14,562	459	9,866	459

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

	Three Months Ended		Nine Months Ended	
	September 30, 2019		September 30, 2019	
Net loss	\$ (17,714 )	)	\$ (41,970 )	)
Adjustments to reconcile net loss to net cash used in operating activities (non-cash items) (1)	5,675		7,132	
Changes in operating assets and liabilities	(733 )	)	(3,976 )	)
Net cash used in operating activities	(12,772 )	)	(38,814 )	)
Net cash provided by investing activities	815		27,211	
Net cash (used in) provided by financing activities	(6,414 )	)	80,377	)
Impact of foreign exchange on cash and restricted cash	(264 )	)	(266 )	)

Net (decrease) increase in cash, cash equivalents and restricted cash	(18,635 )	68,508
Cash, cash equivalents and restricted cash at beginning of period	95,641	8,498
Cash, cash equivalents and restricted cash at end of period	\$ 77,006	\$ 77,006

(1) Primarily related to a \$4.0 million loss on the transfer of non-financial assets during the third quarter of 2019.

**X4 PHARMACEUTICALS, INC.**

**CONDENSED CONSOLIDATED BALANCE SHEETS**

(in thousands)

(Unaudited)

	September 30, 2019	December 31, 2018
<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
<b>Total assets</b>	<b>\$ 109,537</b>	<b>\$ 9,944</b>
<b>Liabilities, convertible preferred stock, redeemable common stock and stockholders' equity (deficit)</b>		
Current liabilities:		
Accounts payable, accrued expenses and other current liabilities	\$ 8,279	\$ 6,220
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, net	19,986	8,145
Lease and other non-current liabilities	2,165	622
Total liabilities	30,430	21,621
Convertible preferred stock	—	64,675
Redeemable common stock	—	734

Total stockholders' equity (deficit)	79,107	(77,086 )
<b>Total liabilities, convertible preferred stock, redeemable common stock and stockholders' equity (deficit)</b>	<b>\$ 109,537</b>	<b>\$ 9,944</b>

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Source: X4 Pharmaceuticals, Inc.

**Investors:**

Stephanie Carrington  
Westwicke, an ICR company  
646-277-1282  
[Stephanie.Carrington@icrinc.com](mailto:Stephanie.Carrington@icrinc.com)

**Media:**

Darcie Robinson  
Westwicke, an ICR company  
203-919-7905  
[Darcie.Robinson@icrinc.com](mailto:Darcie.Robinson@icrinc.com)