



X4 Pharmaceuticals Reports Third Quarter 2022 Financial Results and Provides Corporate Update

November 3, 2022

Following positive mavorixafor Phase 1b data release in multiple chronic neutropenic disorders, X4 continues to look forward to top-line results from its pivotal Phase 3 trial in WHIM syndrome in the fourth quarter of 2022

Conference call to be hosted today at 8:30 a.m. ET

BOSTON, Nov. 03, 2022 (GLOBE NEWSWIRE) -- [X4 Pharmaceuticals](#) (Nasdaq: XFOR), a leader in the discovery and development of novel small-molecule therapeutics to benefit people with diseases of the immune system, today reported financial results for the third quarter ended September 30, 2022 and highlighted recent and upcoming expected milestones.

"This is a truly exciting time for X4 as we near the announcement of top-line data from our first pivotal clinical trial and continue to advance our lead candidate, mavorixafor, towards commercialization," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "Following our September release of positive data from the Phase 1b clinical trial of mavorixafor across a number of chronic neutropenic disorders, we now eagerly await the results from the 4WHIM registration trial. With no oral therapeutic approved for these patients, we believe mavorixafor, if approved, could represent a new opportunity to transform the treatment landscape and create a new standard of care for almost 50,000 underserved patients in the U.S."

Recent Highlights & Anticipated Upcoming Milestones

- In July, X4 completed a private investment in public equity (PIPE) financing, receiving aggregate gross proceeds of approximately \$55 million; the financing included participation from new and existing investors.
- In July, the company announced a strategic re-prioritization of resources towards advancing mavorixafor in chronic neutropenic disorder indications, including WHIM syndrome, while progressing oncology programs only upon completion of strategic partnership(s); the announced strategic update was also inclusive of cost-cutting initiatives estimated to extend X4's cash runway into the third quarter of 2023.
- In late September, X4 held an event highlighting new positive data from its Phase 1b clinical trial demonstrating the ability of mavorixafor to increase and normalize absolute neutrophil counts (ANC) in people with idiopathic, cyclic, or congenital chronic neutropenia (CN) as monotherapy or concurrently with injectable granulocyte colony-stimulating factor (G-CSF). The company believes that these results suggest an expanded market opportunity for mavorixafor that could include almost 50,000 additional diagnosed patients in the U.S.
- The Phase 1b trial in chronic neutropenic disorders is currently being amended and expanded into a Phase 1b/Phase 2 clinical trial to assess the long-term durability, safety, and tolerability of mavorixafor in a larger patient population; X4 anticipates the amended trial to begin generating additional clinical data in the first half of 2023.
- The company recently announced the appointment of industry veteran Mark Baldry to the position of Chief Commercial Officer. In this key role, Mr. Baldry will lead all pre-commercial and product launch efforts for mavorixafor as clinical development advances.
- Following recent completion of the last patient/last visit in the company's Phase 3 clinical trial of mavorixafor in WHIM syndrome (the 4WHIM trial), X4 continues to anticipate the announcement of top-line results from the trial in the fourth quarter of 2022 and the submission for U.S. regulatory approval of mavorixafor in WHIM early in the second half of 2023, if the data are positive.

X4 today also announced that its Chief Medical Officer, Dr. Diego Cadavid, intends to depart the company by the end of 2022, but will continue to support X4 as a consultant. Dr. Cadavid, still a practicing neurologist, is leaving to pursue an opportunity that closely aligns with his commitment to patients with intractable CNS disorders.

X4 is appointing Dr. Murray Stewart as interim Chief Medical Officer while it conducts a search for a permanent replacement for Dr. Cadavid. Dr. Stewart, who has served as a member of the X4 Board of Directors since March 2019, has had a distinguished career in the life sciences industry, including an 18-year tenure at GlaxoSmithKline (GSK), where he held multiple research and development leadership roles, including Chief Medical Officer, Clinical Head of the Biopharma Unit, and Therapy Area Head for metabolic and cardiovascular diseases. Dr. Stewart also previously served as Chief Medical Officer of Rhythm Pharmaceuticals, leading the successful NDA submission and approval of Imcivree™ (setmelanotide), an innovative treatment for rare causes of obesity.

Dr. Stewart commented: "I am very pleased to be able to support X4 through this exciting time, as we prepare to unblind the mavorixafor Phase 3 WHIM data and advance to an NDA submission if the data are positive. I look forward to partnering with the clinical and executive management teams

to continue X4's journey towards providing people with WHIM and other CN disorders with a potentially transformative new therapy."

Third Quarter 2022 Financial Results

- **Cash, Cash Equivalents & Restricted Cash:** X4 had \$81.1 million in cash, cash equivalents, and restricted cash as of September 30, 2022. X4 believes that it has sufficient funds to support company operations into the third quarter of 2023.
- **Research and Development (R&D) Expenses** were \$14.1 million for the third quarter of 2022 as compared to \$13.2 million for the comparable period in 2021. R&D expenses include \$0.6 million and \$0.6 million of certain non-cash expenses for the third quarter of 2022 and 2021, respectively.
- **Selling, General, and Administrative Expenses (SG&A)** were \$6.0 million for the third quarter of 2022 as compared to \$5.9 million for the comparable period in 2021. SG&A expenses include \$0.5 million and \$0.9 million of certain non-cash expenses for the third quarter of 2022 and 2021, respectively.
- **Net Loss:** X4 reported a net loss of \$21.6 million for the third quarter of 2022, as compared to \$20.2 million for the comparable period in 2021. Net losses include \$1.1 million and \$1.5 million of certain non-cash expenses for the third quarter of 2022 and 2021, respectively.

Conference Call and Webcast

X4 will host a conference call and webcast today at 8:30 am ET to discuss important upcoming milestones, including the pending release of results from the company's Phase 3 trial of its lead candidate, mavorixafor, in the treatment of WHIM syndrome. The conference call can be accessed by dialing 1-855-327-6837 within the United States or 1-631-891-4304 internationally, followed by the conference ID: 10020371. The live webcast can be accessed on the investor relations section of X4 Pharmaceuticals' website at www.x4pharma.com. Following the completion of the call, a webcast replay of the conference call will be available on the website

About X4 Pharmaceuticals

X4 Pharmaceuticals is a late-stage clinical biopharmaceutical company leading the discovery and development of novel therapies for people with diseases of the immune system. Our lead clinical candidate is mavorixafor, a first-in-class, small molecule antagonist of chemokine receptor CXCR4 that is being developed as a once-daily oral therapy. Due to mavorixafor's ability to antagonize CXCR4 and improve the mobilization of white blood cells, we believe that mavorixafor has the potential to provide therapeutic benefit across a wide variety of immune system diseases, including a range of chronic neutropenic disorders and certain types of cancer. The efficacy and safety of mavorixafor are being evaluated in a global Phase 3 clinical trial in patients with WHIM syndrome, a rare, primary immunodeficiency disease typically caused by genetic mutations in the CXCR4 receptor gene. We are also studying mavorixafor in two Phase 1b clinical trials – one in patients with chronic neutropenic disorders including congenital, idiopathic, and cyclic neutropenia, and one concurrently with ibrutinib in patients with Waldenström's macroglobulinemia (WM), a rare B-cell lymphoma. Further clinical development of mavorixafor in WM will now be subject to completing a strategic partnership as we focus our resources on advancing mavorixafor for the benefit of patients with chronic neutropenic disorders. We continue to leverage our insights into CXCR4 biology at our corporate headquarters in Boston, Massachusetts and at our research facility in Vienna, Austria. For more information, please visit our website at 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. These statements may be identified by the words "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target," or other similar terms or expressions that concern X4's expectations, strategy, plans, or intentions. Forward-looking statements include, without limitation, statements regarding the clinical development of X4's pipeline and its research and development programs, including the timing of the Phase 3 clinical trial of mavorixafor in WHIM syndrome and the Phase 1b trial in chronic neutropenic disorders; the anticipated reporting of data; interactions with regulators and the timing thereof, anticipated timing of submission for U.S. regulatory approval of mavorixafor in WHIM; expectations regarding the potential efficacy and commercial potential of mavorixafor; and expectations regarding X4's cash runway. Any forward-looking statements in this press release are based on management's current expectations and beliefs. Actual events or results may differ materially from those expressed or implied by any forward-looking statements contained herein, including, without limitation, on account of uncertainties inherent in the initiation and completion of clinical trials and clinical development; the risk that trials and studies may not have satisfactory outcomes; the risk that the outcomes of preclinical studies or earlier clinical trials will not be predictive of later clinical trial results; the risk that initial or interim results from a clinical trial may not be predictive of the final results of the trial or the results of future trials; the potential adverse effects arising from the testing or use of mavorixafor or other product candidates; the risk that the FDA may not support and accept a regulatory submission for mavorixafor, and X4's interactions with the FDA may not have satisfactory outcomes; the risks related to X4's ability to raise additional capital; and other risks and uncertainties, including those described in the section entitled "Risk Factors" in X4's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on August 4, 2022, 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 new events or circumstances, except as required by law.

(Tables Follow)

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,	
	2022	2021	2022	2021
Operating expenses:				
Research and development	\$ 14,110	\$ 13,188	42,044	38,485

Selling, general and administrative	6,044	5,931	20,457	17,567
Gain on sale of non-financial asset	—	—	(509)	—
Total operating expenses	20,154	19,119	61,992	56,052
Loss from operations	(20,154)	(19,119)	(61,992)	(56,052)
Other expense, net	(1,445)	(1,054)	(2,757)	(2,423)
Loss before provision for income taxes	(21,599)	(20,173)	(64,749)	(58,475)
Provision for income taxes	(13)	2	14	14
Net loss	(21,586)	(20,175)	(64,763)	(58,489)
Deemed dividend due to Class B warrant price reset	(287)	—	(2,546)	(8,239)
Net loss attributable to common stockholders	<u>\$ (21,873)</u>	<u>\$ (20,175)</u>	<u>\$ (67,309)</u>	<u>\$ (66,728)</u>
Net loss per share attributable to common stockholders- basic and diluted	<u>\$ (0.26)</u>	<u>\$ (0.76)</u>	<u>\$ (1.32)</u>	<u>\$ (2.71)</u>
Weighted average common shares outstanding-basic and diluted	83,211	26,609	50,976	24,667

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

	Nine months ended September 30,	
	2022	2021
Net loss	\$ (64,763)	\$ (58,489)
Adjustments to reconcile net loss to net cash used in operating activities	6,493	6,809
Changes in operating assets and liabilities	261	(172)
Net cash used in operating activities	<u>(58,009)</u>	<u>(51,852)</u>
Net cash used in investing activities	<u>(69)</u>	<u>(602)</u>
Net cash provided by financing activities	<u>56,586</u>	<u>49,675</u>
Impact of foreign exchange on cash, cash equivalents and restricted cash	<u>(468)</u>	<u>(203)</u>
Net decrease in cash, cash equivalents and restricted cash	(1,960)	(2,982)
Cash, cash equivalents and restricted cash at beginning of period	83,108	80,702
Cash, cash equivalents and restricted cash at end of period	<u>\$ 81,148</u>	<u>\$ 77,720</u>

X4 PHARMACEUTICALS, INC.
CONSOLIDATED BALANCE SHEETS
(in thousands)
(unaudited)

	September 30, 2022	December 31, 2021
Current assets:		
Cash and cash equivalents	\$ 79,855	\$ 81,787
Research and development incentive receivable	909	747
Prepaid expenses and other current assets	4,954	5,344
Total current assets	<u>85,718</u>	<u>87,878</u>
Property and equipment, net	1,194	1,514
Goodwill	17,351	17,351
Right-of-use assets	7,606	8,710
Other assets	1,537	1,723
Total assets	<u><u>\$ 113,406</u></u>	<u><u>\$ 117,176</u></u>
Current liabilities:		
Accounts payable	\$ 3,800	\$ 4,283
Accrued expenses	9,128	7,870
Current portion of lease liability	1,164	1,075
Current portion of long-term debt	14,621	795
Total current liabilities	<u>28,713</u>	<u>14,023</u>
Long-term debt, including accretion, net of discount	18,733	33,139
Lease liabilities	3,799	4,776
Other liabilities	312	826
Total liabilities	<u>51,557</u>	<u>52,764</u>
Total stockholders' equity	<u>61,849</u>	<u>64,412</u>
Total liabilities and stockholders' equity	<u><u>\$ 113,406</u></u>	<u><u>\$ 117,176</u></u>

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Source: X4 Pharmaceuticals