



X4 Pharmaceuticals Reports First-Quarter 2023 Financial Results and Provides Corporate Update

May 4, 2023

Company to host webinar event on May 16th presenting new clinical data from the 4WHIM Phase 3 trial; oral presentation to follow on May 21st at the 2023 Clinical Immunology Society (CIS) Annual Meeting

U.S. NDA submission of mavorixafor for WHIM syndrome on track for early 2H 2023

Presentation of data from ongoing Phase 2 chronic neutropenia trial and clarity on regulatory path forward anticipated in 2Q/3Q 2023

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

BOSTON, May 04, 2023 (GLOBE NEWSWIRE) -- [X4 Pharmaceuticals](#) (Nasdaq: XFOR), a leader in the discovery and development of novel small-molecule therapeutics to benefit people with rare diseases of the immune system, today reported financial results for the first quarter ended March 31, 2023 and highlighted key upcoming expected milestones.

"This is an exciting time at X4 as we look forward to delivering on several expected major milestones throughout the rest of 2023," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "As we recently announced, we will be presenting additional data on secondary endpoints, including results on infection burden among other outcomes metrics, from the Phase 3 trial of mavorixafor in those diagnosed with WHIM syndrome at the upcoming annual meeting of the Clinical Immunology Society; a May 16 company webinar with clinical experts will be followed by an oral data presentation on May 21 at the conference. Additionally, we continue to anticipate submission of our first U.S. New Drug Application for mavorixafor early in the second half of the year. We are also continuing to advance mavorixafor through a Phase 2 clinical trial in people with certain chronic neutropenic disorders and expect to provide clinical and regulatory path updates in the second or third quarter of this year."

Recent and Key Anticipated Upcoming Milestones

Additional Data from the Phase 3 4WHIM Trial to be Presented at Upcoming CIS Meeting:

- In late 2022, the company announced that its Phase 3 clinical trial evaluating once-daily, oral mavorixafor in people with WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) syndrome met its primary endpoint and first key secondary endpoint, with mavorixafor achieving statistically significant and clinically relevant longer times above threshold levels for both absolute neutrophil and absolute lymphocyte counts versus placebo and demonstrating good tolerability in the trial.
- Subsequently, X4 announced that its submitted late-breaker abstract, entitled "*Results of a Phase 3 Trial of an Oral CXCR4 Antagonist, Mavorixafor, for Treatment of Patients With WHIM Syndrome*," was accepted for oral presentation at the Annual Meeting of the Clinical Immunology Society (CIS), which is taking place May 18-21, 2023, in St. Louis, MO.
 - Dr. Raffaele Badolato, Professor of Pediatrics at the University of Brescia (Italy) and an investigator in the 4WHIM clinical trial, will present at 11:30 am CT on Sunday, May 21. Although the session will only be accessible live to conference attendees, X4 will post the slides on its website concurrent with the presentation.

X4 to Host May 16th Webinar Highlighting Infection Data from Phase 3 4WHIM Clinical Trial:

- The company will be hosting a virtual event to present and discuss new data from the 4WHIM Phase 3 clinical trial at 4:00 pm ET on Tuesday, May 16, 2023, following the expected publication of the accepted CIS abstracts.
- New data to be presented during the event will focus on additional secondary endpoints, including the impact of mavorixafor on the rate, severity, and duration of infections, among other outcomes metrics, as measured during the 52-week trial period. Registration for the event can be completed [here](#).
- The event will include commentary from several WHIM patients and from a diverse panel of global clinical experts who manage those with WHIM syndrome and/or certain chronic neutropenic disorders. These experts include:
 - **Charlotte Cunningham-Rundles, MD, PhD**, The David S. Gottesman Professor, Departments of Medicine and Pediatrics, The Prism Immunology Institute, The Icahn School of Medicine at Mount Sinai;
 - **Jean Donadieu, MD, PhD**, Pediatrician, Hemato Oncologic Department of Trousseau Hospital, Coordinator of the French Histiocytosis Registry, Coordinator of the pediatric branch of the French Histiocytosis Reference Center;

- o **Peter E. Newburger, MD**, Editor, *Pediatric Blood & Cancer*, Professor of Pediatrics and Molecular, Cell, and Cancer Biology, UMass Chan Medical School;
 - o **Akiko Shimamura, MD, PhD**, Professor of Pediatrics, Harvard Medical School and Director, Bone Marrow Failure and Myelodysplastic Syndrome Program, Boston Children's Hospital; and
 - o **Teresa K. Tarrant, MD, FAACAP**, Associate Professor of Medicine, Duke University School of Medicine, Director, Clinical Immunology Laboratory DUHS, Vice Chief of Translational Research for Rheumatology and Immunology.
- During the May 16th event, the company also expects to provide an update on its U.S. regulatory activities in support of its U.S. New Drug Application submission for mavorixafor for the treatment of WHIM syndrome, which continues to be expected early in the second half of 2023.

Advancing Mavorixafor in Chronic Neutropenic Disorders:

- In September 2022, X4 presented positive data from a Phase 1b clinical trial demonstrating the ability of one dose of oral mavorixafor to increase and normalize absolute neutrophil counts (ANC) in people with idiopathic, cyclic, or congenital chronic neutropenia as monotherapy or administered concurrently with injectable granulocyte colony-stimulating factor (G-CSF).
- The Phase 1b trial has been expanded into a Phase 2 clinical trial to assess the long-term durability, safety, and tolerability of oral mavorixafor in a larger population with idiopathic, cyclic, or congenital chronic neutropenia. Participants are currently being enrolled in this Phase 2 trial and X4 anticipates announcing clinical data and clarity on the scope and timing of the expected Phase 3 clinical program for mavorixafor in these Chronic Neutropenic disorders in the second or third quarter of 2023.
- X4 also announced today that its abstract, entitled "*Incidence of Serious Infection Events in People With Chronic Neutropenia—Analysis of Real-World Data From Patients in the United States*," was accepted for poster presentation at CIS on Saturday, May 20, 2023 at 1:30 pm CT. This poster will be added to the publications section of the X4 website at the time of the presentation.

First Quarter 2023 Financial Results

- **Cash, Cash Equivalents & Restricted Cash:** X4 had \$94.4 million in cash, cash equivalents, and restricted cash as of March 31, 2023. X4 believes that it has sufficient funds to support company operations into the second quarter of 2024.
- **Research and Development (R&D) Expenses** were \$22.1 million for the first quarter ended March 31, 2023 as compared to \$14.1 million for the comparable period in 2022. R&D expenses for the first quarter ended March 31, 2023 included \$0.8 million of certain non-cash expenses and a \$5.0 million accrual for an in-licensing milestone payment that the company deems probable of occurring.
- **Selling, General and Administrative Expenses (SG&A)** were \$7.2 million for the first quarter ended March 31, 2023 as compared to \$7.7 million for the comparable period in 2022. SG&A expenses included \$0.8 million of certain non-cash expenses for the first quarter ended March 31, 2023.
- **Net Loss:** X4 reported a net loss of \$24.0 million for the first quarter ended March 31, 2023, as compared to \$22.0 million for the comparable period in 2022. Net loss included \$1.6 million of stock-based compensation expense and a \$5.4 million gain for the change in fair value of the company's Class C warrant liability for the first quarter ended March 31, 2023.

Conference Call and Webcast

X4 will host a conference call and webcast today at 8:30 am ET to discuss these financial results and business highlights. The conference call can be accessed by dialing 1-855-327-6837 from the United States or 1-631-891-4304 internationally, followed by the conference ID: 10021600. 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 focused on the discovery and development of novel therapies for people with rare diseases of the immune system. Our lead clinical candidate is mavorixafor, a small molecule antagonist of chemokine receptor CXCR4 that is being developed as an oral, once-daily therapy. Due to its ability to increase the mobilization of mature, functional white blood cells from the bone marrow into the bloodstream, we believe that mavorixafor has the potential to provide therapeutic benefit across a variety of chronic neutropenic disorders, including WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) syndrome, a rare, primary immunodeficiency. Following announcement of positive top-line data from our global, pivotal, 4WHIM Phase 3 clinical trial, we are currently preparing a U.S. regulatory submission seeking approval of oral, once-daily mavorixafor in the treatment of people aged 12 years and older with WHIM syndrome. We are also currently evaluating mavorixafor in a Phase 2 clinical trial in people with certain chronic neutropenic disorders following positive results from a Phase 1b clinical trial of mavorixafor in people with congenital, idiopathic, or cyclic neutropenia. We continue to leverage our insights into CXCR4 and immune system biology at our corporate headquarters in Boston, Massachusetts and at our research center of excellence 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 applicable securities laws, including 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 progress of X4's pipeline development programs; the status of clinical trials, including, without limitation, expectations regarding the data that are being presented, the

expected timing of data releases and evaluation, as well as completion of clinical trials and the timing thereof; interactions with regulators and the timing thereof, including the anticipated NDA submission for U.S. regulatory approval of mavorixafor in WHIM; market opportunities for X4's product candidates; expectations regarding the potential efficacy and commercial potential of mavorixafor; and the sufficiency of X4's cash resources 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 Food and Drug Administration (FDA) may not support and accept a regulatory submission for mavorixafor, and that X4's interactions with the FDA may not have satisfactory outcomes; the risks related to X4's ability to raise additional capital; the impacts of general macroeconomic and geopolitical conditions, including the COVID-19 pandemic, the conflict in Ukraine, rising inflation, and uncertain credit and financial markets on X4's business, clinical trials and financial position; and other risks and uncertainties, including those described in the section entitled "Risk Factors" in X4's Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on March 21, 2023, 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	
	March 31,	
	2023	2022
Operating expenses:		
Research and development	\$ 22,063	\$ 14,113
Selling, general and administrative	7,241	7,664
Gain on sale of non-financial asset	—	(509)
Total operating expenses	29,304	21,268
Loss from operations	(29,304)	(21,268)
Other income (expense), net	5,288	(674)
Loss before provision for income taxes	(24,016)	(21,942)
Provision for income taxes	4	23
Net loss	(24,020)	(21,965)
Deemed dividend due to Class B warrant price reset	—	(2,259)
Net loss attributable to common stockholders	\$ (24,020)	\$ (24,224)
Net loss per share attributable to common stockholders- basic and diluted	\$ (0.16)	\$ (0.72)
Weighted average common shares outstanding-basic and diluted	145,967	33,737

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

	Three Months Ended March 31,	
	2023	2022
Net loss	\$ (24,020)	\$ (21,965)
Adjustments to reconcile net loss to net cash used in operating activities	(3,108)	2,036
Changes in operating assets and liabilities	616	(300)
Net cash used in operating activities	(26,512)	(20,229)
Net cash used in investing activities	(9)	(22)
Net cash (used in) provided by financing activities	(2,124)	4,956
Impact of foreign exchange on cash, cash equivalents and restricted cash	50	(69)
Net decrease in cash, cash equivalents and restricted cash	(28,595)	(15,364)
Cash, cash equivalents and restricted cash at beginning of period	123,028	83,108
Cash, cash equivalents and restricted cash at end of period	\$ 94,433	\$ 67,744

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

	<u>March 31, 2023</u>	<u>December 31, 2022</u>
Current assets:		
Cash and cash equivalents	\$ 93,406	\$ 121,718
Research and development incentive receivable	658	1,152
Prepaid expenses and other current assets	4,158	5,807
Total current assets	98,222	128,677
Property and equipment, net	986	1,104
Goodwill	17,351	17,351
Right-of-use assets	6,844	7,229
Other assets	1,002	1,225
Total assets	\$ 124,405	\$ 155,586
Current liabilities:		
Accounts payable	\$ 5,992	\$ 7,777
Accrued expenses	11,762	12,034
Current portion of lease liability	1,175	1,198
Current portion of long-term debt	554	1,315
Total current liabilities	19,483	22,324
Long-term debt, including accretion, net of discount	31,827	32,304
Lease liabilities	3,377	3,603
Other liabilities	18,042	23,304
Total liabilities	72,729	81,535
Total stockholders' equity	51,676	74,051
Total liabilities and stockholders' equity	\$ 124,405	\$ 155,586

Contacts:

Daniel Ferry (investors)
Managing Director, LifeSci Advisors
daniel@lifesciadvisors.com
(617) 430-7576

Cherilyn Cecchini, M.D. (media)
LifeSci Communications
ccecchini@lifescicomms.com



Source: X4 Pharmaceuticals