X4 Pharmaceuticals Reports Second-Quarter 2023 Financial Results, Provides Corporate Updates, and Reports Emerging Data from Chronic Neutropenia Clinical Program

August 10, 2023

Submission of first U.S. New Drug Application for mavorixafor in WHIM syndrome on track for early 2H 2023

Emerging data from ongoing Phase 2 trial in certain chronic neutropenic disorders show mavorixafor durably increased neutrophil counts and enabled reductions in G-CSF dosing

New market research confirms significant initial target population for mavorixafor given high unmet needs within U.S. chronic neutropenia (CN) market

Plans underway for initiation of Phase 3 trial in certain chronic neutropenic disorders in 1H 2024

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

BOSTON, Aug. 10, 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 second quarter ended June 30, 2023, highlighted key recent and upcoming expected milestones, and presented emerging data from its ongoing Phase 2 clinical trial of mavorixafor in people with certain chronic neutropenic disorders.

“After a highly productive second quarter, X4 continues its forward progress as we fast approach our first U.S. regulatory submission for mavorixafor,” said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. “Importantly, our continued positive momentum enabled us to raise funds through both equity and debt financings. With a current cash position of more than $160 million, we believe our strong balance sheet positions us well and provides us great financial flexibility as we continue to advance our commercial and clinical efforts.”

Dr. Ragan continued: “In addition, we are excited to report today emerging data from participants in our ongoing Phase 2 clinical trial of mavorixafor in certain chronic neutropenic disorders. As our market research continues to suggest significant unmet medical needs and a market poorly served by current injectable therapies, we are quite pleased that the emerging data from our ongoing Phase 2 trial are showing that oral mavorixafor treatment durably increases neutrophil counts, and that these increases have enabled physicians to reduce G-CSF dosing in their patients while maintaining concurrent mavorixafor treatment. We expect to be able to share additional data from this trial in the fourth quarter of this year as we advance towards initiating a pivotal Phase 3 clinical trial in chronic neutropenia in the first half of 2024.”

“Lastly,” Dr. Ragan concluded, “with the recent announcement that Dr. Christophe Arbet-Engels will be joining X4 as our new Chief Medical Officer, we believe we are not only strengthening our readiness for our potential commercial launch of mavorixafor in WHIM syndrome, but also supporting the future growth potential of mavorixafor in chronic neutropenia and other potential indications.”

Recent and Key Anticipated Upcoming Milestones

Advancing Mavorixafor in WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) Syndrome:

- Additional clinical data from the Phase 3 4WHIM trial were presented at a company webinar in May 2023 and at the Annual Meetings of both the Clinical Immunology Society (CIS) and the European Hematology Association (EHA) in May and June 2023, respectively.
- The 4WHIM data presented in the second quarter of 2023 revealed that mavorixafor treatment resulted in statistically significant reductions in annualized infection rates versus placebo and effected clinically meaningful reductions in both the severity and duration of infections versus placebo in trial participants. These data followed disclosure in late 2022 that the 4WHIM trial had met its primary endpoint and first key secondary endpoint and was well tolerated during the trial.
- X4 remains on track for its first U.S. regulatory submission seeking approval of oral, once-daily mavorixafor for the treatment of people aged 12 years and older with WHIM syndrome early in the second half of 2023.
- Given the possibility of U.S. approval and launch of mavorixafor in WHIM syndrome in the first half of 2024, X4 intends to provide an update on its commercial readiness, physician outreach, and potential WHIM market during the fourth quarter of 2023.

***Advancing Mavorixafor in Chronic Neutropenic Disorders**:*

- Given the demonstration of mavorixafor’s ability to durably raise absolute neutrophil counts (ANC) and reduce infection burden in the 4WHIM trial, X4 is currently evaluating mavorixafor for the treatment of certain chronic neutropenic disorders.
- Building on the success of its Phase 1b trial, X4 is now enrolling a Phase 2 clinical trial (NCT04154488) evaluating the

durability, safety, and tolerability of chronic dosing of once-daily oral mavorixafor with or without concurrent treatment with injectable granulocyte colony-stimulating factor (G-CSF) in people with idiopathic, cyclic, and congenital chronic neutropenia.
- Today, X4 reported positive preliminary Phase 2 results from three participants receiving G-CSF and once-daily oral mavorixafor showing robust increases in ANC, maintenance of ANC levels in the normal range, and the ability to reduce G-CSF dose earlier than anticipated. Additional data from this ongoing Phase 2 trial are expected to be shared in the fourth quarter of 2023.
- X4 is planning to initiate a Phase 3 clinical trial evaluating mavorixafor in certain chronic neutropenic disorders in the first half of 2024.
- X4 also announced today that new research into the estimated 50,000 individuals in the U.S. diagnosed with chronic neutropenia confirms significant unmet medical needs exist despite the availability and use of G-CSF and suggests a potential minimal addressable market for mavorixafor of approximately one third of this population, or approximately 15,000 individuals in the U.S., plus meaningful potential market expansion opportunities.

Other Corporate Updates:

- **Additional Mavorixafor Patent Issued:** X4 also announced today the strengthening of its patent portfolio with a third patent covering mavorixafor's composition of matter granted in June 2023. This patent protects compositions of matter comprising mavorixafor and a related substance formed during the mavorixafor manufacturing process through December 2038.
- **Management Team Strengthened with Addition of New Chief Medical Officer:** X4 recently announced the appointment of Christophe Arbet-Engels, MD, PhD, as its new Chief Medical Officer. Dr. Arbet-Engels brings more than 25 years of experience to the company in strategic global drug discovery, development, and commercialization, as well as expertise in rare and orphan diseases.

Recent Financial Events and Second Quarter 2023 Results

- **PIPE Financing Completed**: In May 2023, X4 completed a private investment in public equity (PIPE) financing priced at-the-market, raising approximately $65 million in gross proceeds. Participants in the financing included both new and existing life science investors.
- **X4 Added to Russell 3000® Index**: X4 was added to the broad-market Russell 3000 Index at the conclusion of the 2023 Russell indexes annual reconstitution in late June 2023. The annual reconstitution captures the 4,000 largest U.S. stocks as of April 28, 2023, ranking them by total market capitalization. Membership in the U.S. all-cap Russell 3000 Index remains in place for one year.
- **Completed a $115 million Debt Facility with Hercules Capital**: Subsequent to the end of the second quarter, in early August 2023, X4 announced an expanded $115 million debt facility with Hercules Capital, with an initial drawdown of $22.5 million at closing.
- **Cash, Cash Equivalents, & Restricted Cash**: X4 had $142.3 million in cash, cash equivalents, restricted cash, and marketable securities as of June 30, 2023. Including the $22.5 million drawn down at the closing of the expanded debt facility with Hercules Capital, X4 believes it has sufficient funds to support company operations into 2025 and notes that this projected runway does not include additional potential drawdowns from the debt facility nor the potential monetization of a Priority Review Voucher the company would expect to receive should mavorixafor be approved for WHIM syndrome in the U.S.
- **Research and Development (R&D) Expenses** were $15.6 million for the second quarter ended June 30, 2023 as compared to $13.8 million for the comparable period in 2022. R&D expenses for the second quarter ended June 30, 2023 included $1.1 million of certain non-cash expenses.
- **Selling, General, and Administrative Expenses (SG&A)** were $10.2 million for the second quarter ended June 30, 2023 as compared to $6.7 million for the comparable period in 2022. SG&A expenses for the second quarter ended June 30, 2023 included $1.0 million of certain non-cash expenses.
- **Net Loss**: X4 reported a net loss of $55.7 million for the second quarter ended June 30, 2023, as compared to $21.2 million for the comparable period in 2022. Net losses in the current period include $29.9 million of non-cash adjustments to the fair value of the Company's Class C warrants, which are classified as a liability and are adjusted to fair value each reporting period. Net losses also included $2.1 million of stock-based compensation expense.

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 1-877-451-6152 from the United States or 1-201-389-0879 internationally, followed by the conference ID: 13739751. The live webcast and slide presentation 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 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 designing and planning a Phase 3 clinical program evaluating mavorixafor in certain chronic neutropenic disorders following positive results from a Phase 1b clinical trial and encouraging preliminary results from an ongoing Phase 2 clinical trial 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 development and therapeutic potential of mavorixafor for the treatment of WHIM syndrome, chronic neutropenic disorders and other potential indications; expectations regarding timing for reporting data from ongoing clinical studies or the initiation of future clinical trials, including the timing of reporting additional data from X4’s ongoing Phase 2 trial of mavorixafor in certain chronic neutropenic disorders and the timing of commencing a Phase 3 trial; anticipated regulatory progress, including the submission of a New Drug Application for mavorixafor in WHIM syndrome and the timing thereof; expectations regarding the commercial potential of mavorixafor; estimated market opportunities, including estimated initial target population for mavorixafor within the U.S. chronic neutropenia market; expected duration of patent protection; and X4’s financial position and ability to execute on the next phase of its strategy. 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 earlier clinical trials will not be predictive of later clinical trial results; the uncertainties and timing of the regulatory approval process, including whether the New Drug Application for mavorixafor in WHIM syndrome will be accepted by the FDA; the risk that patient prevalence or market opportunity estimates may be inaccurate; the risks related to X4’s ability to raise additional capital; the impacts of general macroeconomic and geopolitical conditions, 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 Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on May 4, 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 June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Operating expenses:				
Research and development	$ 15,601	$ 13,821	$ 37,664	$ 27,934
Selling, general and administrative	10,204	6,749	17,445	14,413
Gain on sale of non-financial asset	—	—	—	(509)
Total operating expenses	25,805	20,570	55,109	41,838
Loss from operations	(25,805)	(20,570)	(55,109)	(41,838)
Other expense, net	(29,892)	(638)	(24,604)	(1,312)
Loss before provision for income taxes	(55,697)	(21,208)	(79,713)	(43,150)
Provision for income taxes	15	4	19	27
Net loss	(55,712)	(21,212)	(79,732)	(43,177)
Deemed dividend due to Class B warrant price reset	—	—	—	(2,259)
Net loss attributable to common stockholders	$ (55,712)	$ (21,212)	$ (79,732)	$ (45,436)
Net loss per share attributable to common stockholders- basic and diluted	$ (0.33)	$ (0.60)	$ (0.51)	$ (1.31)
Weighted average common shares outstanding-basic and diluted	168,738	35,437	157,416	34,592

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

	Six Months Ended June 30,	
	2023	2022
Net loss	$ (79,732)	$ (43,177)
Adjustments to reconcile net loss to net cash used in operating activities	29,633	4,083
Changes in operating assets and liabilities	2,162	391
Net cash used in operating activities	(47,937)	(38,703)
Net cash used in investing activities	(4,893)	(60)
Net cash provided by financing activities	67,214	4,609
Impact of foreign exchange on cash, cash equivalents and restricted cash	44	(271)
Net increase (decrease) in cash, cash equivalents and restricted cash	14,428	(34,425)
Cash, cash equivalents and restricted cash at beginning of period	123,028	83,108
Cash, cash equivalents and restricted cash at end of period	$ 137,456	$ 48,683

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

	June 30, 2023	December 31, 2022
Current assets:		
Cash and cash equivalents	$ 136,428	$ 121,718
Marketable securities	4,881	—
Research and development incentive receivable	820	1,152
Prepaid expenses and other current assets	5,520	5,807
Total current assets	147,649	128,677
Property and equipment, net	868	1,104
Goodwill	17,351	17,351
Right-of-use assets	6,452	7,229
Other assets	1,079	1,225
Total assets	$ 173,399	$ 155,586
Current liabilities:		
Accounts payable	$ 5,108	$ 7,777
Accrued expenses	15,231	12,034
Current portion of lease liability	1,148	1,198
Current portion of long-term debt	764	1,315
Total current liabilities	22,251	22,324
Long-term debt, including accretion, net of discount	31,836	32,304
Lease liabilities	3,124	3,603
Warrant liability	47,179	23,131
Other liabilities	1,474	173
Total liabilities	105,864	81,535
Total stockholders' equity	67,535	74,051
Total liabilities and stockholders' equity	$ 173,399	$ 155,586

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

Brett Whelan (media)
LifeSci Communications
bwhelan@lifescicomms.com

Source: X4 Pharmaceuticals