X4 Pharmaceuticals Reports Fourth-Quarter and Full-Year 2022 Financial Results and Provides Corporate Update

March 21, 2023

Upcoming expected milestones for investigational therapy mavorixafor in WHIM syndrome include: presentation of additional Phase 3 clinical data in 2Q 2023, NDA submission in early

2H 2023, and potential U.S. launch in 1H 2024

Announcement of data from ongoing chronic neutropenia Phase 2 trial and clarity on regulatory path forward planned in 2Q or 3Q 2023

Conference call to be hosted today at 9:00 a.m. ET

BOSTON, March 21, 2023 (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 fourth quarter and full year ended December 31, 2022 and highlighted key 2022 events and expected upcoming milestones.

"We could not be more pleased with the progress we made in 2022 advancing our investigational therapy, mavorixafor, towards commercialization," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "Our clinical trial data continue to speak for themselves, demonstrating mavorixafor's ability to elevate circulating levels of neutrophils, lymphocytes, and monocytes in people with immune system dysfunction. Following the positive results from our 4WHIM clinical trial in late 2022, we continue to expect presentation of additional Phase 3 data in the second quarter of 2023 and the submission of our first New Drug Application (NDA) in the U.S. early in the second half of the year. We are now enrolling patients in our Phase 2 trial in people with certain chronic neutropenic (CN) disorders and anticipate announcing clinical data from this trial along with clarity on the potential regulatory path forward for mavorixafor in CN disorders in the second or third quarter of 2023."

2022 Highlights & Key Anticipated Upcoming Milestones

Positive Clinical Results: With a tightened focus on advancing mavorixafor in chronic neutropenia indications, X4 announced successful conclusion of and positive data from key clinical programs in 2022:

- **Pivotal, Phase 3 4WHIM trial:** In late November, the company announced that its Phase 3 clinical trial evaluating 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.
    - Additional data from the 4WHIM trial are expected to be presented at medical conferences in the second quarter of 2023.
    - The company is now preparing to meet with U.S. regulatory authorities to discuss next steps in advancing mavorixafor towards an NDA submission, which is still anticipated early in the second half of 2023.
    - To the best of the company's knowledge, mavorixafor is the first and only oral therapy in development to demonstrate durable improvements in severe chronic neutropenia and lymphopenia, the hallmarks of WHIM syndrome, a rare primary immunodeficiency for which there are no approved treatments.
- **Phase 1b Chronic Neutropenia Trial**: 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 company continues to believe that these results suggest an expanded future market opportunity for mavorixafor that could include up to 50,000 diagnosed patients in the U.S. with certain forms of chronic neutropenia.
    - The Phase 1b trial has now been amended and 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 the Phase 2 CN trial and X4 anticipates announcing clinical data and clarity on the scope and timing of the expected Phase 3 clinical program for mavorixafor in chronic neutropenia in the second or third quarter of 2023.

Greater Insight into Mavorixafor Market Potential: Throughout 2022, X4 presented results from both its clinical and research programs, further supporting the commercial potential of mavorixafor in a variety of chronic neutropenia indications, including WHIM syndrome, at multiple, prominent medical conferences.

- Conferences included: the American Academy of Allergy, Asthma & Immunology (AAAAI) Annual Meeting, the Clinical Immunology Society (CIS) Annual Meeting, the European Hematology Association (EHA) Annual Congress, the North American Immuno-Hematology Clinical Education & Research (NICER) Symposium, the European Society for Immunodeficiencies (ESID), the National Organization for Rare Disorders (NORD) Fall Summit, and the Annual Meeting of the American Society of Hematology (ASH).
- These presentations not only highlighted new insights into the genotype and phenotype of people with WHIM syndrome, helping to identify additional patients and educate treating physicians, but also spotlighted new understandings into and confirmation of mavorixafor's ability to mobilize mature, functional white blood cells from the bone marrow into the blood, and helped elucidate the unmet medical needs and the size of the U.S. population living with severe chronic neutropenia.

Strong Balance Sheet to Support Drive to Commercialization: Despite continued challenging market conditions, during 2022, X4 successfully completed two large financings, raising aggregate gross proceeds of approximately $120 million and has subsequently re-negotiated its loan facility agreement:

- In July 2022, X4 completed a private investment in public equity (PIPE) financing, receiving aggregate gross proceeds of approximately $55 million.
- In December 2022, the company completed a public offering that yielded gross proceeds of approximately $65.0 million before deducting underwriting discounts and estimated offering expenses and any proceeds from the exercise of the warrants to be issued in the offering.
- On January 6, 2023, X4 entered into a Second Amended and Restated Loan and Security Agreement with Hercules Capital that amended and restated all previous loan and security agreements, and that included the extension of the interest-only payment period through the third quarter of 2024 and an agreement to further extend the interest-only period to the first quarter of 2026 if certain milestones are achieved.
- In addition, the covenant under the Hercules agreement requiring that the company maintain cash in an aggregate amount greater than $30.0 million was lowered to $20.0 million, subject to certain terms and conditions.

Fourth Quarter and Full Year 2022 Financial Results

- **Cash, Cash Equivalents & Restricted Cash:** X4 had $123.0 million in cash, cash equivalents, and restricted cash as of December 31, 2022. X4 believes that it has sufficient funds to support company operations into the second quarter of 2024.
- **Research and Development (R&D) Expenses** were $19.0 million and $61.1 million for the fourth quarter and full year ended December 31, 2022, respectively, as compared to $12.2 million and $50.6 million for the comparable periods in 2021. R&D expenses included $0.5 million and $2.5 million of certain non-cash expenses for the fourth quarter and full year ended December 31, 2022, respectively.
- **Selling, General and Administrative Expenses (SG&A)** were $6.6 million and $27.0 million for the fourth quarter and full year ended December 31, 2022, respectively, as compared to $7.1 million and $24.7 million for the comparable periods in 2021. SG&A expenses included $0.6 million and $2.7 million of certain non-cash expenses for the fourth quarter and full year ended December 31, 2022, respectively.
- **Net Loss:** X4 reported a net loss of $29.1 million and $93.9 million for the fourth quarter and full year ended December 31, 2022, respectively, as compared to $30.2 million and $88.7 million for the comparable periods in 2021. Net loss included $1.1 million and $5.2 million of stock-based compensation expenses for the fourth quarter and full year ended December 31, 2022, respectively. Net loss included $1.6 million and $6.2 million of stock-based compensation expenses for the fourth quarter and full year ended December 31, 2021, respectively. Net loss for the fourth quarter and full year ended December 31, 2021 included a non-cash goodwill impairment charge of $9.8 million. There was no goodwill impairment charge in 2022.

Conference Call and Webcast

X4 will host a conference call and webcast today at 9:00 am ET to discuss these financial results and business highlights. The conference call can be accessed by dialing 1-855-327-6837 within the United States or 1-631-576-4098 internationally, followed by the conference ID: 10020997. 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 company 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 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 macroeconomic 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 December 31,		Year Ended December 31,	
	2022	2021	2022	2021
Operating expenses:				
Research and development	$ 19,014	$ 12,162	$ 61,058	$ 50,647
Selling, general and administrative	6,563	7,135	27,020	24,702
Gain on sale of non-financial asset	—	—	(509)	—
Goodwill impairment	—	9,758	—	9,758
Total operating expenses	25,577	29,055	87,569	85,107
Loss from operations	(25,577)	(29,055)	(87,569)	(85,107)
Other expense, net	(3,513)	(1,149)	(6,270)	(3,572)
Loss before provision for income taxes	(29,090)	(30,204)	(93,839)	(88,679)
Provision for income taxes	14	3	28	17
Net loss	(29,104)	(30,207)	(93,867)	(88,696)
Deemed dividend due to Class B warrant price reset	—	(5,704)	(2,546)	(13,943)
Net loss attributable to common stockholders	$ (29,104)	$ (35,911)	$ (96,413)	$ (102,639)
Net loss per share attributable to common stockholders- basic and diluted	$ (0.29)	$ (1.24)	$ (1.52)	$ (3.99)
Weighted average common shares outstanding-basic and diluted	100,766	29,010	63,526	25,749

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

	Year ended December 31,	
	2022	2021
Net loss	$ (93,867)	$ (88,696)
Adjustments to reconcile net loss to net cash used in operating activities	11,029	19,289
Changes in operating assets and liabilities	5,736	(1,498)
Net cash used in operating activities	(77,102)	(70,905)

Net cash used in investing activities	(103)	(615)
Net cash provided by financing activities	117,230	74,245
Impact of foreign exchange on cash, cash equivalents and restricted cash	(105)	(319)
Net decrease in cash, cash equivalents and restricted cash	39,920	2,406
Cash, cash equivalents and restricted cash at beginning of period	83,108	80,702
Cash, cash equivalents and restricted cash at end of period	$ 123,028	$ 83,108

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

	December 31, 2022	December 31, 2021
Current assets:		
Cash and cash equivalents	$ 121,718	$ 81,787
Research and development incentive receivable	1,152	747
Prepaid expenses and other current assets	5,807	5,344
Total current assets	128,677	87,878
Property and equipment, net	1,104	1,514
Goodwill	17,351	17,351
Right-of-use assets	7,229	8,710
Other assets	1,226	1,723
Total assets	$ 155,586	$ 117,176
Current liabilities:		
Accounts payable	$ 7,777	$ 4,283
Accrued expenses	12,034	7,870
Current portion of lease liability	1,198	1,075
Current portion of long-term debt	1,315	795
Total current liabilities	22,324	14,023
Long-term debt, including accretion, net of discount	32,304	33,139
Lease liabilities	3,603	4,776
Other liabilities	23,304	826
Total liabilities	81,535	52,764
Total stockholders' equity	74,051	64,412
Total liabilities and stockholders' equity	$ 155,586	$ 117,176

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