# X4 Pharmaceuticals Reports Fourth-Quarter and Full-Year 2023 Financial Results and Provides Corporate Update

March 21, 2024

*Launch preparations underway in anticipation of possible U.S. approval of mavorixafor for WHIM syndrome; U.S. PDUFA target action date set for April 30, 2024*

*Additional Phase 2 clinical data and initiation of global Phase 3 clinical trial of mavorixafor for the treatment of certain chronic neutropenic disorders expected in 1H24*

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

BOSTON, March 21, 2024 (GLOBE NEWSWIRE) -- **X4 Pharmaceuticals** (Nasdaq: XFOR), a company driven to improve the lives of people with rare diseases of the immune system, today reported financial results for the fourth quarter and full year ended December 31, 2023, and highlighted key 2023 events and expected upcoming milestones.

"Following an incredibly productive 2023, we are expecting a transformative year in 2024," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "In 2023, we not only successfully submitted our New Drug Application for the potential first and only treatment for WHIM syndrome in the U.S., but also achieved clinical proof of concept for oral, once daily mavorixafor to address chronic neutropenia (CN), a second rare immunodeficiency. The excitement at X4 is palpable as we approach the potential launch of our first product and the expected initiation of the pivotal CN clinical program, both of which would bring us one step closer to fulfilling our mission to make progress for those diagnosed with rare diseases of the immune system and few or no treatment options."

**2023 Highlights and Expected Milestones on Mavorixafor Clinical & Commercial Development**

- In September 2023, X4 announced the submission of a New Drug Application (NDA) to the United States Food and Drug Administration (FDA) for the approval of once-daily, oral mavorixafor to treat individuals aged 12 and older with WHIM syndrome, a rare, combined primary immunodeficiency and chronic neutropenic disorder named for its four main manifestations: Warts, Hypogammaglobulinemia, Infections, and Myelokathexis.
- In October 2023, the FDA accepted for filing and granted Priority Review of the mavorixafor NDA in WHIM syndrome, establishing a goal of six months review from the date of acceptance and assigning a Prescription Drug User Fee Act (PDUFA) target action date of April 30, 2024. Company discussions with the FDA to date have indicated that this action date remains on track.
- The company continues to build out its rare disease field force and supportive commercial infrastructure as it prepares for the potential launch of mavorixafor for WHIM syndrome in the U.S., if approved.
- For the WHIM syndrome indication, mavorixafor has been granted Breakthrough Therapy Designation, Fast Track Designation, and Rare Pediatric Disease (RPD) Designation in the U.S., and Orphan Drug Status in both the U.S. and European Union. Upon FDA approval of a product with RPD designation, the sponsor can receive a Priority Review Voucher that can be used to obtain priority review for a subsequent application or sold to another drug sponsor.
- X4 continues to advance its ex-U.S. commercialization strategy for mavorixafor, evaluating multiple global and regional opportunities for partnerships or direct sales, including geographies where the company could leverage its U.S. approval, if received.
- Following multiple presentations of positive Phase 1b and preliminary Phase 2 clinical trial data on mavorixafor in the treatment of certain chronic neutropenic disorders, the company continues to expect to announce additional Phase 2 results in 15+ trial participants and to initiate a global, pivotal, Phase 3 clinical trial in certain CN indications in the first half of 2024.

**Fourth-Quarter and Full-Year 2023 Financial Results**

- **Cash, Cash Equivalents, Restricted Cash, and Short-Term Marketable Securities:** X4 had $115.2 million in cash, cash equivalents, restricted cash, and marketable securities as of December 31, 2023. 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 its 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 $15.3 million and $72.0 million for the fourth quarter and full year ended December 31, 2023, respectively, as compared to $19.0 million and $61.1 million for the comparable periods in 2022. R&D expenses included $1.1 million and $4.4 million of certain non-cash expenses for the fourth quarter and full year ended December 31, 2023, respectively.
- **Selling, General and Administrative Expenses (SG&A)** were $9.9 million and $35.5 million for the fourth quarter and full year ended December 31, 2023, respectively, as compared to $6.6 million and $27.0 million for the comparable periods in 2022. SG&A expenses included $1.4 million and $4.3 million of certain non-cash expenses for the fourth quarter and full year ended December 31, 2023, respectively.
- **Net Loss:** X4 reported a net loss of $19.1 million and $101.2 million for the fourth quarter and full year ended December 31, 2023, respectively, as compared to $29.1 million and $93.9 million for the comparable periods in 2022. Net loss included $2.5 million and $8.7 million of stock-based compensation expenses for the fourth quarter and full year ended December 31, 2023, respectively. 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.

**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 within the United States or 1-201-389-0879 internationally, followed by the conference ID: 13744107. 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 driven to improve the lives of people with rare diseases of the immune system. Our lead clinical candidate is mavorixafor, a selective, small-molecule antagonist of chemokine receptor CXCR4 that is being developed as an oral, once-daily therapy across a variety of immunodeficiencies, including WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) syndrome and certain chronic neutropenic disorders. Following successful completion of a global, pivotal, Phase 3 clinical trial, we are seeking U.S. regulatory approval of oral, once-daily mavorixafor for the treatment of people aged 12 years and older with WHIM syndrome. We are also currently planning a Phase 3 clinical program evaluating mavorixafor in certain chronic neutropenic disorders. 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 PDUFA for U.S. regulatory approval of mavorixafor in WHIM and the priority review voucher; 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 grant approval 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 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 expected to be filed with the Securities and Exchange Commission (SEC) on March 21, 2024, 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.

**X4 PHARMACEUTICALS, INC.**
**CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS**
**(in thousands, except per share amounts)**
**(unaudited)**

| | Three Months Ended December 31, | | Year Ended December 31, | |
|---|---|---|---|---|
| | **2023** | **2022** | **2023** | **2022** |
| Operating expenses: | | | | |
| Research and development | $ 15,272 | $ 19,014 | $ 72,017 | $ 61,058 |
| Selling, general and administrative | 9,927 | 6,563 | 35,505 | 27,020 |
| Gain on sale of non-financial asset | — | — | — | (509) |
| Total operating expenses | 25,199 | 25,577 | 107,522 | 87,569 |
| Loss from operations | (25,199) | (25,577) | (107,522) | (87,569) |
| Other income (expense), net | 6,102 | (3,513) | 6,433 | (6,270) |
| Loss before provision for income taxes | (19,097) | (29,090) | (101,089) | (93,839) |

| | | | | |
|---|---|---|---|---|
| Provision for income taxes | 33 | 14 | 78 | 28 |
| Net loss | (19,130) | (29,104) | (101,167) | (93,867) |
| Deemed dividend due to Class B warrant price reset | — | — | — | (2,546) |
| Net loss attributable to common stockholders | $ (19,130) | $ (29,104) | $ (101,167) | $ (96,413) |
| Net loss per share attributable to common stockholders- basic and diluted | $ (0.10) | $ (0.29) | $ (0.57) | $ (1.52) |
| Weighted average common shares outstanding-basic and diluted | 198,766 | 100,766 | 177,812 | 63,526 |

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

| | Year ended December 31, | |
|---|---|---|
| | **2023** | **2022** |
| Net loss | $ (101,167) | $ (93,867) |
| Adjustments to reconcile net loss to net cash used in operating activities | 4,311 | 11,029 |
| Changes in operating assets and liabilities | 344 | 5,736 |
| Net cash used in operating activities | (96,512) | (77,102) |
| Net cash used in investing activities | (14,883) | (103) |
| Net cash provided by financing activities | 88,516 | 117,230 |
| Impact of foreign exchange on cash, cash equivalents and restricted cash | 99 | (105) |
| Net decrease in cash, cash equivalents and restricted cash | (22,780) | 39,920 |
| Cash, cash equivalents and restricted cash at beginning of period | 123,028 | 83,108 |
| Cash, cash equivalents and restricted cash at end of period | $ 100,248 | $ 123,028 |

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

| | **December 31, 2023** | **December 31, 2022** |
|---|---|---|
| Current assets: | | |
| Cash and cash equivalents | $ 99,216 | $ 121,718 |
| Marketable securities | 15,000 | — |
| Research and development incentive receivable | 562 | 1,152 |
| Prepaid expenses and other current assets | 7,298 | 5,807 |
| Total current assets | 122,076 | 128,677 |
| Property and equipment, net | 745 | 1,104 |
| Goodwill | 17,351 | 17,351 |
| Right-of-use assets | 5,650 | 7,229 |
| Other assets | 1,436 | 1,225 |
| **Total assets** | $ 147,258 | $ 155,586 |
| Current liabilities: | | |
| Accounts payable | $ 8,947 | $ 7,777 |
| Accrued expenses | 12,816 | 12,034 |
| Current portion of lease liability | 1,099 | 1,198 |
| Current portion of long-term debt | — | 1,315 |
| Total current liabilities | 22,862 | 22,324 |
| Long-term debt, including accretion, net of discount | 54,570 | 32,304 |
| Lease liabilities | 2,612 | 3,603 |
| Other liabilities | 16,115 | 23,304 |
| Total liabilities | 96,159 | 81,535 |
| Total stockholders' equity | 51,099 | 74,051 |
| **Total liabilities and stockholders' equity** | $ 147,258 | $ 155,586 |

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Source: X4 Pharmaceuticals