X4 Pharmaceuticals Reports Third-Quarter 2023 Financial Results and Provides Corporate Updates

November 9, 2023

U.S. New Drug Application for mavorixafor in WHIM syndrome accepted for Priority Review, establishing a PDUFA target action date of April 30, 2024; X4 eligible to receive Priority Review Voucher if approved

Presentations of additional data from ongoing Phase 2 clinical trial of mavorixafor in chronic neutropenia expected in 4Q 2023 and 1H 2024

Pivotal, global Phase 3 trial of mavorixafor in chronic neutropenia expected to initiate in 1H 2024

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

BOSTON, Nov. 09, 2023 (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 third quarter ended September 30, 2023 and highlighted key recent and upcoming expected milestones.

"We could not be more pleased with the significant milestones we've achieved in the clinical development of mavorixafor this year," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "Following the recent FDA acceptance of our New Drug Application seeking approval of mavorixafor for the treatment of WHIM syndrome, we are now preparing for a possible U.S. launch in the second quarter of 2024. With key learnings gleaned from our ongoing Phase 2 trial in chronic neutropenia and input from the FDA, we have now finalized the design of our planned pivotal, global Phase 3 trial evaluating mavorixafor in certain chronic neutropenic disorders and expect to initiate in the first half of 2024."

Recent and Key Anticipated Milestones

Advancing Mavorixafor in WHIM Syndrome:

- In late October 2023, the United States Food and Drug Administration (FDA) accepted for filing X4's New Drug Application (NDA) for once-daily, oral mavorixafor to treat individuals aged 12 and older with WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) syndrome, a rare, primary immunodeficiency. The FDA granted Priority Review of the mavorixafor NDA, 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. The FDA has notified X4 that it does not currently plan to hold an advisory committee meeting to review the filing.
- Due to mavorixafor's Rare Pediatric Disease designation for WHIM syndrome in the U.S., X4 is eligible to receive a Priority Review Voucher (PRV) that can be used to obtain priority review for a subsequent application or can be sold to another drug sponsor, should mavorixafor be approved by the FDA for WHIM syndrome.
- In anticipation of a potential second quarter 2024 U.S. launch of mavorixafor in WHIM syndrome, X4 has continued to build out its go-to market organization, with key hires across commercial and medical functions, increased interactions with key stakeholders and rare disease patient advocacy organizations, and has launched a disease-awareness campaign called What If It's WHIM? aimed at furthering the understanding of WHIM syndrome and educating patients and physicians on the importance and benefits of early diagnosis.

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

- Following an earlier presentation of preliminary data from the first 3 participants in the company's ongoing Phase 2 trial evaluating the safety and efficacy of mavorixafor in certain chronic neutropenic disorders, the company anticipates presenting additional longer-term treatment data on these participants in a poster session at the upcoming 65th Annual Meeting of the American Society of Hematology (ASH), taking place December 9-12, 2023 in San Diego, CA.
- In addition, X4 plans to present efficacy and safety data from at least 15 of the currently enrolled participants in the Phase 2 chronic neutropenia trial during the first half of 2024.
- The design of a planned pivotal, global Phase 3 clinical trial evaluating the safety and efficacy of mavorixafor in the treatment of people 12 years and older with idiopathic or congenital neutropenia is now complete and incorporates FDA input. X4 anticipates initiation of this Phase 3 trial in the first half of 2024.

Other Recent Corporate Highlights:

- **Management Team Strengthened with Addition of New Chief Medical Officer:** In August 2023, X4 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.

- **Completed a $115 million Debt Facility with Hercules Capital**: In August 2023, X4 announced an expanded $115 million debt facility with Hercules Capital, with an initial drawdown of $22.5 million at closing.
- **Expanded Company Board of Directors:** In October 2023, X4 announced the addition of industry veteran R. Keith Woods to its Board of Directors bringing significant sales and commercial strategy experience most recently at argenx, where he helped build a global enterprise focused on developing treatments for rare autoimmune diseases and successfully launch the company's flagship product, Vyvgart®.

Third-Quarter 2023 Results

- **Cash, Cash Equivalents, Restricted Cash, and Short-Term Marketable Securities:** X4 had $142.7 million in cash, cash equivalents, restricted cash, and marketable securities as of September 30, 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 $19.1 million for the third quarter ended September 30, 2023 as compared to $14.1 million for the comparable period in 2022. R&D expenses for the third quarter ended September 30, 2023 included $1.3 million of certain non-cash expenses.
- **Selling, General, and Administrative Expenses (SG&A)** were $8.1 million for the third quarter ended September 30, 2023 as compared to $6.0 million for the comparable period in 2022. SG&A expenses for the third quarter ended September 30, 2023 included $1.1 million of certain non-cash expenses.
- **Net Loss:** X4 reported a net loss of $2.3 million for the third quarter ended September 30, 2023, as compared to $21.6 million for the comparable period in 2022. Net losses in the current period include a non-cash gain of $25.2 million related to the company's Class C warrant liability, which is adjusted to fair value each reporting period. Net losses also included $2.4 million of stock-based compensation expense.

Conference Call and Webcast

The company will host a conference call and webcast today at 8:30 a.m. ET. 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: 13741400. The live webcast will be accessible through the investor relations section of X4 Pharmaceuticals' website at www.x4pharma.com. Following the completion of the call, a webcast replay will be available on the 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 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 timing and potential impact of FDA acceptance and priority review of X4's NDA for mavorixafor for the treatment of individuals with WHIM syndrome; the commercial launch of mavorixafor, if approved; 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; expectations regarding the commercial potential of mavorixafor; and X4's financial position, potential cash runway, 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, risks of obtaining and maintaining regulatory approvals, including, but not limited to, potential regulatory delays or rejections or the risk that the FDA will require additional trials or data; 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 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 August 10, 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.

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,	
	2023	**2022**	**2023**	**2022**
Operating expenses:				
Research and development	$ 19,081	$ 14,110	$ 56,745	$ 42,044
Selling, general and administrative	8,133	6,044	25,578	20,457
Gain on sale of non-financial asset	—	—	—	(509)
Total operating expenses	27,214	20,154	82,323	61,992
Loss from operations	(27,214)	(20,154)	(82,323)	(61,992)
Other expense, net	24,935	(1,445)	331	(2,757)
Loss before provision for income taxes	(2,279)	(21,599)	(81,992)	(64,749)
Provision for income taxes	26	(13)	45	14
Net loss	(2,305)	(21,586)	(82,037)	(64,763)
Deemed dividend due to Class B warrant price reset	—	(287)	—	(2,546)
Net loss attributable to common stockholders	$ (2,305)	$ (21,873)	$ (82,037)	$ (67,309)
Net loss per share attributable to common stockholders- basic and diluted	$ (0.01)	$ (0.26)	$ (0.48)	$ (1.32)
Weighted average common shares outstanding-basic and diluted	196,988	83,211	170,751	50,976

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

	Nine Months Ended September 30,	
	2023	**2022**
Net loss	$ (82,037)	$ (64,763)
Adjustments to reconcile net loss to net cash used in operating activities	7,570	6,493
Changes in operating assets and liabilities	5,702	261
Net cash used in operating activities	(68,765)	(58,009)
Net cash used in investing activities	(10,050)	(69)
Net cash provided by financing activities	88,419	56,586
Impact of foreign exchange on cash, cash equivalents and restricted cash	(28)	(468)
Net increase (decrease) in cash, cash equivalents and restricted cash	9,576	(1,960)
Cash, cash equivalents and restricted cash at beginning of period	123,028	83,108
Cash, cash equivalents and restricted cash at end of period	$ 132,604	$ 81,148

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

	September 30, 2023	December 31, 2022
Current assets:		
Cash and cash equivalents	$ 131,581	$ 121,718
Marketable securities	10,102	—
Research and development incentive receivable	393	1,152
Prepaid expenses and other current assets	5,796	5,807
Total current assets	147,872	128,677
Property and equipment, net	772	1,104
Goodwill	17,351	17,351
Right-of-use assets	6,054	7,229
Other assets	1,244	1,225
Total assets	$ 173,293	$ 155,586

Current liabilities:		
Accounts payable	$ 8,132	$ 7,777
Accrued expenses	16,184	12,034
Current portion of lease liability	1,116	1,198
Current portion of long-term debt	—	1,315
Total current liabilities	25,432	22,324
Long-term debt, including accretion, net of discount	54,322	32,304
Lease liabilities	2,848	3,603
Warrant liability	22,014	23,131
Other liabilities	1,083	173
Total liabilities	105,699	81,535
Total stockholders' equity	67,594	74,051
Total liabilities and stockholders' equity	$ 173,293	$ 155,586

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