X4 Pharmaceuticals Reports First-Quarter 2024 Financial Results and Provides Corporate Updates

May 7, 2024

U.S. launch underway for XOLREMDI™ (mavorixafor) in WHIM syndrome

XOLREMDI Phase 3 4WHIM clinical trial data published online in Blood*, the journal of the American Society of Hematology*

Presentation of interim clinical data from the ongoing mavorixafor Phase 2 trial in chronic neutropenia expected at a planned company investor event in June 2024

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

BOSTON, May 07, 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 first quarter ended March 31, 2024 and highlighted key recent and upcoming expected milestones.

"With the recent U.S. approval of our first commercial product, XOLREMDI (mavorixafor) in WHIM syndrome, we are now deploying our experienced rare disease field force, engaging with payers to communicate the XOLREMDI value proposition, and, most importantly, enabling patient access to treatment as quickly as possible," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "Concurrent with this approval, we received a Rare Pediatric Disease Priority Review Voucher, which we intend to monetize. In addition, we look forward to sharing data in June from our ongoing Phase 2 clinical trial exploring the use of mavorixafor in certain chronic neutropenic disorders."

Recent and Key Anticipated Upcoming Milestones

- In late April 2024, X4 announced that the U.S. Food and Drug Administration (FDA) approved XOLREMDI (mavorixafor) capsules for use in patients 12 years of age and older with WHIM syndrome (warts, hypogammaglobulinemia, infections and myelokathexis) to increase the number of circulating mature neutrophils and lymphocytes. The approval was based on results from the global, pivotal 4WHIM Phase 3 clinical trial that evaluated the efficacy and safety of XOLREMDI in people diagnosed with WHIM syndrome.
- Data from the 4WHIM trial were recently published online in *Blood*, the journal of the American Society of Hematology, and new data from the trial and its open-label extension phase were recently presented at the annual meeting of the Clinical Immunology Society (CIS).
- In June 2024, X4 expects to present interim data from its ongoing Phase 2 clinical trial exploring the use of mavorixafor as a once-daily oral treatment for people with certain chronic neutropenic disorders. Interim data are expected to include hematological results from at least 15 participants treated with fixed doses of either mavorixafor monotherapy or mavorixafor in combination with injectable granulocyte colony-stimulating factor (G-CSF).
- X4 also plans to initiate a global, pivotal Phase 3 clinical trial in the second quarter of 2024 that aims to evaluate the efficacy, safety, and tolerability of oral once-daily mavorixafor (with or without G-CSF) in people with congenital or acquired primary autoimmune and idiopathic chronic neutropenia who are experiencing recurrent and/or serious infections.
- X4 currently expects to seek approval from the European Medicines Agency (EMA) for mavorixafor in WHIM syndrome in late 2024/early 2025.

First-Quarter 2024 Results

- **Cash, Cash Equivalents, Restricted Cash and Short-Term Marketable Securities:** X4 had $81.6 million in cash, cash equivalents, restricted cash, and short-term marketable securities as of March 31, 2024. X4 believes it has sufficient funds to support company operations into 2025 and notes that this projected runway does not include the potential monetization of the Priority Review Voucher the company has received as a result of the U.S. approval of XOLREMDI.
- **Research and Development (R&D) Expenses** were $19.9 million for the first quarter ended March 31, 2024 as compared to $22.1 million for the comparable period in 2023. R&D expenses for the first quarter ended March 31, 2024 included $0.8 million of certain non-cash expenses.
- **Selling, General, and Administrative Expenses (SG&A)** were $17.4 million for the first quarter ended March 31, 2024 as compared to $7.2 million for the comparable period in 2023. SG&A expenses for the first quarter ended March 31, 2024 included $1.0 million of certain non-cash expenses.

- **Net Loss:** X4 reported a net loss of $51.8 million for the first quarter ended March 31, 2024, as compared to $24.0 million for the comparable period in 2023. Net losses in the current period include a non-cash loss of $13.8 million related to the company's Class C warrant liability, which is adjusted to fair value each reporting period. Net losses also included $1.7 million of stock-based compensation expense.

First-quarter 2024 expenses included greater costs due to commercial and medical team expansions as well as launch preparations across the commercial and medical organizations.

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: 13745487. 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 is delivering progress for patients by developing and commercializing innovative therapies for those with rare diseases of the immune system and significant unmet needs. Leveraging our expertise in CXCR4 and immune system biology, we have successfully developed mavorixafor, which has received U.S. approval as XOLREMDI™ (mavorixafor) capsules in its first indication. We are also evaluating the use of mavorixafor in additional potential indications. X4 corporate headquarters are in Boston, Massachusetts and our research center of excellence is 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 X4's plans with respect to the potential to monetize the Priority Review Voucher; X4's expected cash runway; X4's commercialization plans and ongoing efforts with respect to XOLREMDI and the expected timing thereof; and other statements regarding X4's future operations, financial performance, financial position, prospectus, objectives and other future events. 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, unanticipated costs and expenses may be greater than anticipated; the company's cash and cash equivalents may not be sufficient to support its operating plan for as long as anticipated; delays, interruptions or failures in the manufacture and supply of our products; and the company's ability to obtain additional funding to support its clinical development and commercial programs; 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, 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 March 31,	
	2024	**2023**
Operating expenses:		
Research and development	$ 19,854	$ 22,063
Selling, general and administrative	17,435	7,241
Total operating expenses	37,289	29,304
Loss from operations	(37,289)	29,304
Other (expense) income, net	(14,458)	5,288
Loss before provision for income taxes	(51,747)	(24,016)
Provision for income taxes	19	4
Net loss	$ (51,766)	$ (24,020)
Net loss per share attributable to common stockholders- basic and diluted	$ (0.26)	$ (0.16)
Weighted average common shares outstanding-basic and diluted	199,992	145,967

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

	March 31, 2024	December 31, 2023
Current assets:		
Cash and cash equivalents	$ 60,493	$ 99,216
Marketable securities	20,376	15,000
Research and development incentive receivable	702	562
Prepaid expenses and other current assets	5,762	7,298
Total current assets	87,333	122,076
Property and equipment, net	742	745
Goodwill	17,351	17,351
Right-of-use assets	5,264	5,650
Other assets	1,492	1,436
Total assets	$ 112,182	$ 147,258
Current liabilities:		
Accounts payable	$ 8,935	$ 8,947
Accrued expenses	13,473	12,816
Current portion of lease liability	1,133	1,099
Total current liabilities	23,541	22,862
Long-term debt, including accretion, net of discount	54,824	54,570
Lease liabilities	2,318	2,612
Warrant liability	29,438	15,683
Other liabilities	1,025	432
Total liabilities	111,146	96,159
Total stockholders' equity	1,036	51,099
Total liabilities and stockholders' equity	$ 112,182	$ 147,258

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Source: X4 Pharmaceuticals