X4 Pharmaceuticals Reports Second Quarter 2024 Financial Results and Provides Corporate Updates

August 8, 2024

First U.S. commercial sales of XOLREMDI™ (mavorixafor) following April 2024 FDA approval

Positive interim data from Phase 2 trial of mavorixafor in chronic neutropenia; full trial results expected in November 2024

Initiated global, pivotal Phase 3 trial of mavorixafor in chronic neutropenia

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

BOSTON, Aug. 08, 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 second quarter ended June 30, 2024 and highlighted key recent and upcoming expected milestones.

Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals commented on the company's significant accomplishments in the second quarter of 2024: "When we founded X4 ten years ago, we had a vision to advance our lead asset, an orally active CXCR4 antagonist called mavorixafor, to help those with rare diseases and few to no treatment options. This past quarter, we were able to realize this vision, receiving U.S. approval of mavorixafor (as XOLREMDI™) in WHIM syndrome, a rare primary immunodeficiency. The U.S. launch of XOLREMDI is now underway with our commercial team in place and executing on our strategy, and with WHIM patients 12 years and older now being treated with the only therapy indicated for and targeting the underlying cause of their disease."

Dr. Ragan continued: "We also made tremendous progress this quarter in the development of mavorixafor for those with chronic neutropenia, not only demonstrating for the first time the ability of mavorixafor to durably and meaningfully increase neutrophil counts in people living with chronic neutropenia (CN), but also initiating a global, pivotal Phase 3 trial in CN. All of these accomplishments mark a significant step forward for X4, now a fully integrated biopharmaceutical company, as we continue to explore additional uses for and maximize the global potential of mavorixafor for patients."

Recent and Key Anticipated Upcoming Milestones

XOLREMDI™ (mavorixafor) in WHIM Syndrome, a Rare Primary Immunodeficiency:

- **FDA Approval of XOLREMDI.** In 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.
- **U.S. Launch Update**: X4 is currently executing its U.S. launch of XOLREMDI and generating its first revenues from product sales. Launch efforts have included: increasing interactions with targeted physicians and rare disease patient advocacy groups, continuing disease awareness and education campaigns, and activating its X4Connect™ and nurse educator programs, which provide a suite of patient support services.
- **4WHIM Phase 3 Data Publications**. During the second quarter of 2024, data from the company's completed global, pivotal Phase 3 4WHIM clinical trial were published online in *Blood*, the journal of the American Society of Hematology (ASH), and new data from the trial and its open-label extension phase were presented at the annual meeting of the Clinical Immunological Society (CIS).
- **Maximizing the Global Opportunity in WHIM Syndrome**. X4 currently expects to submit for regulatory approval of mavorixafor in WHIM syndrome from the European Medicines Agency (EMA) by early 2025, while also exploring additional potential opportunities in geographies where the company may be able to efficiently leverage its U.S. FDA approval.

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

- **Positive Interim Phase 2 Clinical Data.** In June 2024, X4 presented positive clinical data from its ongoing Phase 2 clinical trial evaluating the safety and efficacy of mavorixafor in the treatment of people with chronic neutropenia (CN). The interim results showed that once-daily oral mavorixafor was generally well tolerated and durably increased participants' absolute neutrophil counts (ANC) both as a monotherapy and in combination with stable doses of injectable granulocyte colony-stimulating factor (G-CSF), the only therapy approved in the U.S. for severe chronic neutropenia.

- **Full Phase 2 Data Expected in November 2024.** Data from the completed Phase 2 CN trial, including data from the group of participants receiving mavorixafor and dose-adjusted G-CSF, are expected to be presented in November 2024.
- **Initiation of Phase 3 4WARD Trial.** In June 2024, the company also announced the initiation of its global, pivotal Phase 3 clinical trial (NCT06056297), evaluating the efficacy, safety, and tolerability of oral, once-daily mavorixafor (with or without stable doses of G-CSF) in people with congenital, acquired primary autoimmune, or idiopathic CN who are experiencing recurrent and/or serious infections. The 52-week 4WARD trial is a randomized, double-blind, placebo-controlled, multicenter study aiming to enroll 150 participants.

Second Quarter 2024 Financial Results

- **Gain on Sale of Non-Financial Asset:** In May 2024, X4 recognized a gain on the sale of a priority review voucher (PRV) to a third party for $105.0 million in cash. The PRV was awarded to X4 by the FDA under its Rare Pediatric Disease program upon the approval of XOLREMDI. Under this program, the FDA awards PRVs to sponsors of rare pediatric disease product applications that meet certain criteria to encourage development of new drugs and biologics for the prevention and treatment of rare pediatric diseases.
- **Cash, Cash Equivalents, Restricted Cash and Short-Term Marketable Securities:** X4 had $169.5 million in cash, cash equivalents, restricted cash, and short-term marketable securities as of June 30, 2024. X4 believes it has sufficient funds to support company operations into late 2025 and notes that this projected runway does not include potential future XOLREMDI revenue.
- **Revenue and Cost of Revenue:** For the three months ended June 30, 2024, X4 reported net product revenue of $0.6 million and cost of revenue of $0.3 million related to the sale of XOLREMDI. Cost of revenue includes approximately $0.2 million of license costs, including sale-based royalties and operational milestones capitalized as an intangible asset and amortized over the life of the underlying intellectual property. X4 did not report product revenue or cost of revenue in any prior period.
- **Research and Development (R&D) Expenses** were $20.9 million for the second quarter ended June 30, 2024 as compared to $15.6 million for the comparable period in 2023. R&D expenses for the second quarter ended June 30, 2024 included $1.2 million of certain non-cash expenses.
- **Selling, General, and Administrative Expenses (SG&A)** were $13.3 million for the second quarter ended June 30, 2024 as compared to $10.2 million for the comparable period in 2023. SG&A expenses for the second quarter ended June 30, 2024 included $1.2 million of certain non-cash expenses.
- **Net Income (Loss):** X4 reported net income of $90.8 million for the second quarter ended June 30, 2024, as compared to a net loss of $55.7 million for the comparable period in 2023. Net income in the current period includes the sale of a PRV for $105.0 million, as noted above, and a non-cash gain of $20.2 million related to the fair value remeasurement of the company's Class C warrant liability.

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 1-800-225-9448 from the United States or 1-203-518-9708 internationally, followed by the conference ID: X4PHARMA. 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 XOLREMDI™ (mavorixafor)
XOLREMDI (mavorixafor) is a selective CXCR4 receptor antagonist approved in the U.S. for use in patients 12 years of age and older with WHIM syndrome to increase the number of circulating mature neutrophils and lymphocytes. CXCR4 receptor stimulation by its ligand, CXCL12, has been shown to play a key role in the movement of white blood cells (leukocytes) to and from the bone marrow compartment. Full prescribing and safety information for XOLREMDI can be found at www.xolremdi.com.

About WHIM Syndrome
WHIM syndrome is a rare, combined primary immunodeficiency and chronic neutropenic disorder caused by CXCR4 receptor dysfunction that results in impaired mobilization of white blood cells from the bone marrow into peripheral circulation. WHIM syndrome is named for its four classic manifestations: warts, hypogammaglobulinemia, infections, and myelokathexis, although only a minority of patients experience all four manifestations in the acronym. People with WHIM syndrome characteristically have low blood levels of neutrophils (neutropenia) and lymphocytes (lymphopenia), and as a result, experience serious and/or frequent infections.

About Chronic Neutropenia and Mavorixafor
Chronic neutropenia is a rare blood condition lasting more than three months, persistently or intermittently, and characterized by increased risk of infections and reduced quality of life due to abnormally low levels of neutrophils circulating in the blood. Neutrophils are retained in the bone marrow by the CXCR4/CXCL12 axis, creating a reserve of cells. Downregulation of the CXCR4 receptor by mavorixafor, an orally active CXCR4 antagonist, has been shown to mobilize neutrophils from the bone marrow into the peripheral blood across multiple disease states. The level of circulating neutrophils is typically measured by drawing blood to determine the absolute neutrophil count (ANC).

About the 4WARD Global, Pivotal, Phase 3 Clinical Trial
The 4WARD trial is a global, pivotal Phase 3 clinical trial (NCT06056297) evaluating the efficacy, safety, and tolerability of oral, once-daily mavorixafor (with or without G-CSF) in people with congenital, acquired primary autoimmune, or idiopathic chronic neutropenia who are experiencing recurrent and/or serious infections. The 52-week trial is a randomized, double-blind, placebo-controlled, multicenter study aiming to enroll 150 participants with confirmed trough ANC levels less than 1,500 cells per microliter at baseline screening and histories of two or more serious and/or recurrent infections in the prior year. The primary endpoint of the trial is based on two outcome measures: annualized infection rate and positive ANC response.

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, implied or express statements regarding 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, prospects, objectives and other future events; the potential therapeutic benefit of mavorixafor; the initiation, timing, progress, and results of our current and future studies and clinical trials, including the Phase 2 clinical trial in chronic neutropenia and the Phase 3 4WARD clinical trial and related preparatory work and the period during which the results of the trials will become available; and the mission and goals for our business. Any forward-looking statements in this press release are based on management's current expectations and beliefs. These forward-looking statements are neither promises nor guarantees of future performance, and are subject to a variety of risks and uncertainties, many of which are beyond X4's control, which could cause actual results to differ materially from those contemplated in these forward-looking statements, including, without limitation, the risks that: unanticipated costs and expenses may be greater than anticipated; X4'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 X4's products; X4's ability to obtain additional funding to support its clinical development and commercial programs; we may encounter adverse events for mavorixafor at any stage that negatively impact development and/or commercialization; the expected availability, content, and timing of clinical data from our ongoing clinical trials of mavorixafor may be delayed or unavailable, including clinical results from our ongoing Phase 2 clinical trial and the announced Phase 3 4WARD trial; the trials and studies may not have satisfactory outcomes; the outcomes of preclinical studies or earlier clinical trials will not be predictive of later clinical trial results; the design and rate of enrollment for clinical trials, including the current design of our Phase 3 clinical trial evaluating mavorixafor in certain chronic neutropenic disorders may not enable successful completion of the trial(s); we may be unable to obtain and maintain regulatory approvals; uncertainties inherent in the initiation and completion of preclinical studies and clinical trials and clinical development; there will be changes in expected or existing competition; there will be changes in the regulatory environment; our business may be adversely affected and our costs may increase if any of our key collaborators fails to perform its obligations or terminates our collaboration; the internal and external costs required for our ongoing and planned activities, and the resulting impact on expense and use of cash, may be higher than expected which may cause us to use cash more quickly than we expect or to change or curtail some of our plans or both; and other risks and uncertainties, including those described in the section entitled "Risk Factors" in X4's Annual Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on May 7, 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.

(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,	
	2024	2023	2024	2023
Product revenue, net	$ 563	$ —	$ 563	$ —
Costs and operating expenses:				
Cost of revenue	268	—	268	—
Research and development	20,914	15,601	40,768	37,664
Selling, general and administrative	13,278	10,204	30,713	17,445
Gain on sale of non-financial asset	(105,000)	—	(105,000)	—
Total operating (income) expense	(70,540)	25,805	(33,251)	55,109
Income (loss) from operations	71,103	(25,805)	33,814	(55,109)
Other income (expense), net:	19,748	(29,892)	5,290	(24,604)
Income (loss) before provision for income taxes	90,851	(55,697)	39,104	(79,713)
Provision for income taxes	18	15	37	19
Net income (loss)	$ 90,833	$ (55,712)	$ 39,067	$ (79,732)
Net income (loss) per share: basic	$ 0.45	$ (0.33)	$ 0.20	$ (0.51)
Weighted average shares--basic	200,440	168,738	200,216	157,416
Net income (loss) per share: diluted	$ 0.45	$ (0.33)	$ 0.19	$ (0.51)
Weighted average shares--diluted	200,801	168,738	200,456	157,416

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

(unaudited)

	June 30, 2024	December 31, 2023
Current assets:		
Cash and cash equivalents	$ 147,218	$ 99,216
Marketable securities	21,536	15,000
Research and development incentive receivable	854	562
Inventory	831	—
Prepaid expenses and other current assets	4,977	7,298
Total current assets	175,416	122,076
Property and equipment, net	808	745
Goodwill	17,351	17,351
Intangible asset, net	10,375	—
Right-of-use assets	4,872	5,650
Other assets	1,789	1,436
Total assets	$ 210,611	$ 147,258
Current liabilities:		
Accounts payable	$ 7,986	$ 8,947
Accrued expenses	19,744	12,816
Current portion of lease liability	1,172	1,099
Total current liabilities	28,902	22,862
Long-term debt, including accretion, net of discount	75,030	54,570
Lease liabilities	2,026	2,612
Warrant liability	9,223	15,683
Other liabilities	979	432
Total liabilities	116,160	96,159
Total stockholders' equity	94,451	51,099
Total liabilities and stockholders' equity	$ 210,611	$ 147,258

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Source: X4 Pharmaceuticals