X4 Pharmaceuticals Reports First Quarter 2025 Financial Results and Provides Corporate Updates

May 1, 2025

4WARD Phase 3 chronic neutropenia trial in full swing;
full enrollment on track for 3Q or 4Q 2025 and top-line data in 2H 2026

Additional analyses of mavorixafor clinical trial data increase confidence in successful outcome of 4WARD trial

XOLREMDI® net U.S. revenues $3.5 million since May 2024 launch

Conference call and webcast today at 8:30 am ET

BOSTON, May 01, 2025 (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, 2025, and highlighted key recent events and expected upcoming milestones.

"The first quarter of 2025 was an extremely productive and value-adding period for X4," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "Not only did we make significant progress in activating sites and enrolling participants in our ongoing mavorixafor Phase 3 trial in chronic neutropenia, but we also continued to support U.S. commercialization of XOLREMDI (mavorixafor) in WHIM syndrome, while also significantly expanding our global potential following acceptance of our EU regulatory submission in WHIM and the announcement of two international commercialization partnerships. We look forward to continuing to deliver on our milestones in the coming year toward our goal of maximizing the potential of mavorixafor to benefit patients with rare immunodeficiencies."

Key First-Quarter 2025 and Recent Corporate Highlights

Advancing Mavorixafor in Chronic Neutropenia (CN)

- **4WARD Trial Updates**. X4 continues to enroll participants in its ongoing global, pivotal Phase 3 clinical trial (NCT06056297), evaluating oral, once-daily mavorixafor in people with congenital, acquired primary autoimmune, or idiopathic CN who are experiencing recurrent and/or serious infections.
  - As previously reported, an amendment to the 4WARD protocol has been implemented, focusing enrollment on those with the highest unmet needs and refining the definition of ANC response, making it consistent across all trial participants. Overall, the trial seeks to demonstrate statistically significant increases in ANC response and corresponding decreases in annualized infection rate between those on mavorixafor versus placebo.
  - To date, the demographics of the enrolled population are balanced and representative of the targeted commercial CN patient populations; baseline absolute neutrophil counts (ANC) and historical infection rates are consistent with this high unmet need population.
  - X4 continues to expect full enrollment in the trial in the third or fourth quarter of 2025, and disclosure of top-line data in the second half of 2026.
- **Further Data Analyses Continue to Increase Confidence in Potential 4WARD Success.** X4 recently applied the 4WARD trial ANC response criteria to individual participant results in the completed mavorixafor Phase 3 4WHIM trial and the Phase 2 CN trial to further assess the likelihood of 4WARD trial success. The company believes that the results of these analyses (details of which are available in the company's updated investor presentation) further support the potential of mavorixafor to not only elevate ANC in the 4WARD trial participants, but that these ANC elevations should correspond with significant reductions in annualized infection rates in the mavorixafor treated trial population.
- **Mavorixafor Patent Update:** In March 2025, X4 received a Notice of Allowance from the U.S. Patent and Trademark Office on its application 17/941,509, which claims include the use of mavorixafor in treating severe chronic, idiopathic, and autoimmune neutropenia in patients without a CXCR4 genetic variation. The patent is expected to expire in the U.S. in March of 2041; similar patent applications are pending in Europe, China, Japan, and Canada.

Commercializing XOLREMDI® (mavorixafor) in WHIM Syndrome, a Rare Primary Immunodeficiency

- **Revenue Update**: X4 has now generated $3.5 million in U.S. sales of XOLREMDI (mavorixafor) from its mid-May 2024 launch through March 2025. The company also disclosed that newly identified patients on treatment are increasing in share of the overall current population on XOLREMDI, demonstrating the positive impact of ongoing WHIM education and

awareness.

- **Upcoming WHIM Clinical and Survey Data Presentations:** Published abstracts accepted for poster presentation at the upcoming meeting of the Clinical Immunology Society (CIS) summarize the following results:
    - *Phase 3 Open Label Extension (OLE) Wart Data*: Two-year data from the OLE phase of the 4WHIM Phase 3 clinical trial evaluating once-daily oral mavorixafor in people with WHIM syndrome revealed marked clinical improvement in wart severity as assessed by Clinical Global Impression of Severity across 70 defined wart areas.
    - *First-Ever WHIM Infection Burden Survey Results*: Of the 20 WHIM patient respondents, 60% of those <18 years and 73% of those ≥18 years reported experiencing at least one infection in the previous 3 months, with 25% requiring overnight hospitalization due to infection. The study concludes that "the frequency and severity of infections requiring medical care and hospitalization underscores the urgency to proactively treat patients with WHIM syndrome."
- **Maximizing the Global Opportunity for Mavorixafor in WHIM Syndrome**:
    - *MAA Acceptance*. In January 2025, X4 announced that its submitted Marketing Authorization Application (MAA) for mavorixafor in the treatment of WHIM syndrome was validated for review by the European Medicines Agency (EMA), enabling possible approval in the first half of 2026.
    - *Ex-US Partnerships*. In the first quarter of 2025, the company announced that it had entered into two ex-U.S. partnerships for the commercialization of mavorixafor: the first, an exclusive licensing and supply agreement under which Norgine Pharma UK will commercialize mavorixafor in Europe, Australia, and New Zealand and the second, with taiba rare to distribute and commercialize XOLREMDI for the treatment of WHIM syndrome in Saudi Arabia, United Arab Emirates, Qatar, Oman, Kuwait, Bahrain, and Egypt following the necessary regulatory approvals in those countries and/or regions.

Strategic Restructuring

In February 2025, X4 announced a strategic restructuring to sharpen focus and maximize the opportunity for mavorixafor in chronic neutropenia while also optimizing its U.S. promotion of XOLREMDI. X4 continues to expect that these efforts will decrease spending by $30-35 million annually.

Reverse Stock Split

On April 24, 2025, X4 announced a one-for-thirty reverse stock split of the company's common stock. The reverse stock split took effect at 12:01 a.m. ET on April 28, 2025, and the company's common stock began trading on a split-adjusted basis on The Nasdaq Capital Market as of the opening of trading the same day under a new CUSIP number (98420X202). The reverse split reduced the number of outstanding shares of the company's Common Stock from approximately 173.6 million shares to approximately 5.8 million shares.

First-Quarter 2025 Financial Results

- **Cash position:** X4 had $87.7 million in cash, cash equivalents, restricted cash, and marketable securities as of March 31, 2025. The company continues to believe it has sufficient funds to support operations into the first half of 2026.
- **Revenue and Cost of Revenue:** For the first quarter ended March 31, 2025, X4 reported net revenue of $28.8 million and cost of revenue of $4.7 million. Revenue in the quarter included license and other revenues of $27.9 million associated with the completed Norgine agreement and XOLREMDI product revenue of $0.9 million. Cost of revenue included $4.5 million of sublicense royalties associated with the Norgine agreement.
- **Research and Development (R&D) Expenses** were $18.5 million for the first quarter ended March 31, 2025, as compared to $19.9 million for the comparable period in 2024. R&D expenses included $0.3 million of certain non-cash expenses for the first quarter ended March 31, 2025.
- **Selling, General, and Administrative (SG&A) Expenses** were $15.0 million for the first quarter ended March 31, 2025, as compared to $17.4 million for the comparable period in 2024. SG&A expenses included $0.3 million of certain non-cash expenses for the first quarter ended March 31, 2025.
- **Net Income (Loss):** X4 reported net income of $0.3 million for the first quarter ended March 31, 2025, as compared to a net loss of $51.8 million for the comparable period in 2024. Net income included $0.5 million of stock-based compensation expenses for the first quarter ended March 31, 2025.

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 1-800-343-4849 from the United States or 1-203-518-9848 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 conclusion of the call, a webcast replay will be available on the website.

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 XOLREMDI® (mavorixafor)
XOLREMDI (mavorixafor) is a selective CXCR4 receptor antagonist approved in the U.S. as a once-daily oral treatment for use in patients 12 years of age and older with WHIM syndrome to increase the number of circulating mature neutrophils and lymphocytes. XOLREMDI is the only treatment specifically approved for patients with WHIM syndrome in the U.S.

About Chronic Neutropenia and Mavorixafor
Chronic neutropenia is a primary, rare blood condition lasting more than three months, persistently or intermittently, and characterized by low levels of circulating neutrophils and 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 functional 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 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 1,000 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 expertise in CXCR4 and immune system biology, X4 has successfully developed mavorixafor, an orally available CXCR4 antagonist that is currently being marketed in the U.S. as XOLREMDI® in its first indication. The company is also evaluating additional uses of mavorixafor and is conducting a global, pivotal Phase 3 clinical trial (4WARD) in people with certain chronic neutropenic disorders. X4 is headquartered in Boston, Massachusetts. For more information, please visit www.x4pharma.com.

X4 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 the initiation, timing, progress, and results of our current and future preclinical studies and clinical trial; the timing and period during which the results of the trials will become available and reported, as well as our research and development programs; market opportunities for X4’s product candidates, including potential benefits from partnerships; expectations regarding the commercial potential of mavorixafor and ongoing engagement and feedback from regulatory authorities; and the sufficiency of X4’s cash resources and pro forma cash resources, including the potential impact of the 2025 strategic restructuring, and expectations regarding X4’s cash runway.

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 the risks that: X4’s restructuring activities may be more costly or time-consuming than expected or may not achieve their intended results and savings; X4’s future financial performance and position may not improve resulting in difficulties in implementing X4’s business strategy, and plans and objectives for future operations; the expected decrease in annual spending could negatively impact X4’s commercial plans and strategy for mavorixafor; the expected sufficiency of X4’s existing cash resources and runway may not be accurate resulting in the need for additional financing sooner than anticipated or unexpected liquidity constraints; the internal and external costs required for X4’s ongoing and planned activities, and the resulting impact on expense and use of cash, may be higher than expected, which may cause the company to use cash more quickly than expected or to change or curtail some of X4’s plans or both; the expected availability, content, and timing of clinical data from X4’s ongoing clinical trials of mavorixafor may be delayed or unavailable, including the ongoing Phase 3 clinical trial; trials, studies and research programs may be delayed and may not have satisfactory outcomes, additionally earlier trials and studies may not be predictive of later trials and studies; the design and rate of enrollment for current clinical trials may not enable successful completion of the trial(s); the commercial opportunity for mavorixafor may be smaller than anticipated; X4 may be unable to obtain and maintain regulatory approvals; adverse safety effects may arise from the testing or use of X4’s product and product candidates; the need to align with collaborators may hamper or delay development and commercialization efforts or increase costs; business may be adversely affected and costs may increase if any of the company’s key collaborators fails to perform its obligations or terminates the collaboration; X4’s may be unable to advance and commercialize and increase sales in mavorixafor to treat chronic neutropenia or to optimize the U.S. promotion of XOLREMDI® (mavorixafor), approved for the treatment of WHIM; and other risks and uncertainties, including those described in the section entitled “Risk Factors” in X4’s most recent Annual Report on X4’s Form 10-K , as well as in other filings X4 makes with the Securities and Exchange Commission, including its quarterly report on Form 10-Q, 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 March 31,	
	2025	**2024**
License and other revenue	$ 27,865	$ —
Product revenue, net	942	—

Total revenue	28,807	—
Costs and operating expenses:		
Cost of revenue	4,716	—
Research and development	18,513	19,854
Selling, general and administrative	15,021	17,435
Total operating expenses	38,250	37,289
Loss from operations	(9,443)	(37,289)
Other income (expense), net	9,759	(14,458)
Income (loss) before provision for income taxes	316	(51,747)
Provision for income taxes	34	19
Net income (loss)	$ 282	$ (51,766)
Net income (loss) per share- basic	$ 0.04	$ (7.77)
Weighted average common shares outstanding- basic	6,840	6,666
Net income (loss) per share- diluted	$ 0.04	$ (7.77)
Weighted average common shares outstanding- diluted	6,869	6,666

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

	March 31, 2025	December 31, 2024
Current assets:		
Cash and cash equivalents	$ 40,257	$ 55,699
Accounts receivable	509	1,070
Marketable securities	46,694	46,361
Research and development incentive receivable	665	640
Inventory	3,089	2,817
Prepaid expenses and other current assets	4,483	5,588
Total current assets	95,697	112,175
Property and equipment, net	487	776
Goodwill	17,351	17,351
Intangible asset, net	9,813	10,000
Right-of-use assets	3,652	4,065
Other assets	3,013	2,080
Total assets	$ 130,013	$ 146,447
Current liabilities:		
Accounts payable	$ 11,115	$ 8,621
Accrued expenses	12,611	23,005
Deferred revenue	855	—
Current portion of lease liability	1,303	1,251
Total current liabilities	25,884	32,877
Long-term debt, including accretion, net of discount	75,629	75,425
Lease liabilities	1,109	1,410
Warrant liability	2,925	13,755
Deferred revenue and other liabilities	1,522	831
Total liabilities	107,069	124,298
Total stockholders' equity	22,944	22,149
Total liabilities and stockholders' equity	$ 130,013	$ 146,447

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