X4 Pharmaceuticals Reports Fourth Quarter and Full Year 2024 Financial Results and Provides Corporate Update

March 25, 2025

Phase 3 4WARD trial in chronic neutropenia now activated at ~90% of targeted trial sites; full enrollment expected in 3Q or 4Q 2025; top-line data anticipated in 2H 2026

4WARD trial protocol refined, increasing confidence in successful outcome

2024 XOLREMDI® net revenues $2.6 million since May launch; company expects ramp up in 2025 as targeted physician outreach increases patient finding and pull-through

Conference call and webcast today at 8:30 am ET

BOSTON, March 25, 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 fourth quarter and full year ended December 31, 2024, and highlighted key 2024 and recent events and expected upcoming milestones.

"As expected, 2024 was a transformative year for the company and our momentum has continued into 2025," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "With the U.S. approval and launch of our first product, XOLREMDI® (mavorixafor) in WHIM syndrome, we are now a fully integrated company advancing our mission to serve those with rare immunodeficiencies and few treatment options. As we await word from the EU regulatory authority on the potential approval of mavorixafor for WHIM in that region, we've continued to expand our potential reach to the global WHIM community through our recently completed commercialization partnerships in the EU, Australia, New Zealand, and the Middle East and North Africa (MENA) territories."

Dr. Ragan continued: "In addition, we continue to make meaningful progress in our efforts to develop mavorixafor for the larger immunodeficiency population with chronic neutropenia (CN). Following positive results from our Phase 2 trial of mavorixafor in certain CN populations, we are currently conducting a global, pivotal Phase 3 trial in CN. With a large number of sites now activated and global screening ongoing, we expect full trial enrollment in the third or fourth quarter of this year and top-line trial data in the second half of 2026."

Dr. Ragan concluded: "Market research and patient testimonials continue to strengthen our belief that there remains significant untapped potential for XOLREMDI for the U.S. and possibly global WHIM patient populations. We are equally confident in mavorixafor's future potential to address unmet needs in the global CN community."

Key 2024 and Recent Corporate Highlights

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

- **FDA Approval and Launch of XOLREMDI:** In May 2024, X4 launched XOLREMDI® (mavorixafor) following approval by the U.S. Food and Drug Administration (FDA) for its 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. Concurrent with the approval of XOLREMDI, X4 received a priority review voucher (PRV), which it subsequently sold to another drug developer for $105 million.
- **U.S. Launch Update:** X4 is continuing to execute on its commercialization of XOLREMDI in the U.S., generating $2.6 million in sales from its mid-May launch through December 2024. During the year, the company advanced disease awareness by engaging with physicians and rare disease patient advocacy groups through a combination of in-person and targeted digital education campaigns. The company's suite of patient services, including its X4Connect™ and nurse educator programs, continue to provide access and support for patients prescribed XOLREMDI.
- **Clinical Data Publications and Presentations:** Journal publications and presentations of clinical data results at top medical meetings, including those of the American Society of Hematology (ASH), the American Academy of Allergy, Asthma, and Immunology (AAAAI), and the Clinical Immunology Society (CIS), have provided further visibility on the XOLRMEDI approval.
- **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), meeting an important corporate milestone. Given a typical 12- to 15-month review process, the company believes approval to be possible in the first half of 2026.

EU/ANZ Partnership. Also in January 2025, the company announced that it had entered into an exclusive licensing and supply agreement under which Norgine Pharma UK will commercialize mavorixafor in Europe, Australia, and New Zealand following any regulatory approvals in those territories. X4 received €28.5 million as an upfront payment and is eligible to receive up to €226 million in potential regulatory and commercial milestone payments in addition to tiered, double-digit royalties.

MENA Partnership. In addition, X4 announced in February 2025, that it had entered into an agreement 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 any approvals in those countries. Pending regulatory approvals in the region, taiba expects to be able to make XOLREMDI available to WHIM patients through a named-patient (compassionate use) program that allows physicians to prescribe medicines approved in other countries to local patients with no other treatment options.

Advancing Mavorixafor in Chronic Neutropenia (CN)

- **Phase 2 Clinical Data De-Risk Ongoing Phase 3 4WARD Clinical Trial.** Throughout 2024, X4 presented positive interim and full data sets from its Phase 2 trial that evaluated mavorixafor in the treatment of people with chronic neutropenia (CN), as a monotherapy and in combination with injectable granulocyte colony-stimulating factor (G-CSF), the only therapy approved in the U.S. for severe chronic neutropenia. The six-month, open-label clinical trial enrolled a total of 23 participants and demonstrated that:
  - Once-daily oral mavorixafor was generally well tolerated +/- G-CSF, with no drug-related serious adverse events reported, consistent with previous clinical studies;
  - Mavorixafor treatment durably and meaningfully increased participants' mean absolute neutrophil counts (ANC) across all study populations; and
  - Physicians were willing and able to reduce the use of G-CSF in participants also treated with mavorixafor, maintaining mean ANC levels in the normal range.
- **Ongoing Phase 3 4WARD Trial.** In June 2024, the company announced the initiation of its global, pivotal Phase 3 clinical trial (NCT06056297), evaluating oral, once-daily mavorixafor (+/- 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.
- **4WARD Trial Updates**. X4 announced today that this first-of-its-kind trial is now activated at ~90% of targeted sites worldwide.

  Refining the 4WARD Protocol: Based on FDA and EMA guidance, an amendment to the 4WARD protocol has been implemented, focusing enrollment on those with the highest unmet needs and refining a co-primary endpoint. The trial is now only enrolling participants with moderate and severe neutropenia (ANC below 1,000 cells per microliter), an ANC level consistent with the company's targeted patient population for mavorixafor, if approved. In addition, the ANC component of the primary endpoint will now be uniform across all participants, seeking to demonstrate infection benefit from an ANC increase of at least 500 cells per microliter in study participants on active drug.

  Enrollment Update: Given the average screening rates currently observed, the company expects the trial to be fully enrolled in the third or fourth quarter of 2025, and disclosure of top-line data in the second half of 2026.

Strategic Restructuring

In February 2025, X4 announced a strategic restructuring to sharpen focus and maximize the opportunity for mavorixafor in chronic neutropenia. Related activities included reducing overall headcount, discontinuing research efforts, pausing pre-clinical drug candidate programs, and closing the company's facility in Vienna, Austria, as well as right-sizing its U.S. commercial field team and streamlining other spending. X4 continues to expect that these efforts will decrease spending by $30-35 million annually.

Fourth-Quarter and Full-Year 2024 Financial Results

- **Cash position:** X4 had $102.8 million in cash, cash equivalents, restricted cash, and marketable securities as of December 31, 2024. Pro-forma for the €28.5 million payment received from Norgine in January 2025 and the expected financial impact of the strategic restructuring announced in February 2025, the company believes it has sufficient funds to support operations into the first half of 2026.
- **Revenue and Cost of Revenue:** For the fourth quarter and full year ended December 31, 2024, X4 reported net product revenue of $1.4 million and $2.6 million, respectively, and cost of revenue of $0.3 million and $0.8 million, respectively, related to sales of XOLREMDI.
- **Research and Development (R&D) Expenses** were $21.7 million and $81.6 million for the fourth quarter and full year ended December 31, 2024, respectively, as compared to $15.3 million and $72.0 million for the comparable periods in 2023. R&D expenses included $1.2 million and $4.3 million of certain non-cash expenses for the fourth quarter and full year ended December 31, 2024, respectively.
- **Selling, General, and Administrative (SG&A) Expenses** were $15.1 million and $61.5 million for the fourth quarter and full year ended December 31, 2024, respectively, as compared to $9.9 million and $35.5 million for the comparable periods

in 2023. SG&A expenses included $1.0 million and $3.9 million of certain non-cash expenses for the fourth quarter and full year ended December 31, 2024, respectively.

- **Gain on Sale of Non-Financial Asset:** For the year ended December 31, 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.
- **Net Loss:** X4 reported a net loss of $39.8 million and $37.5 million for the fourth quarter and full year ended December 31, 2024, respectively, as compared to $19.1 million and $101.2 million for the comparable periods in 2023. Net loss for the year ended December 31, 2024 includes the sale of a PRV for $105.0 million, as noted above. Net loss included $2.2 million and $8.2 million of stock-based compensation expenses for the fourth quarter and full year ended December 31, 2024, 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 1-800-267-6316 from the United States or 1-203-518-9783 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 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 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 its ongoing Phase 3 clinical trial; trials and studies may be delayed and may not have satisfactory outcomes, earlier trials and studies may not be predictive of later trials and studies, including clinical results from the completed Phase 2 clinical trial and research focused on assessing the ability of mavorixafor monotherapy to durably increase absolute neutrophil count in patients with chronic neutropenic; the design and rate of enrollment for clinical trials, including the current design of the ongoing Phase 3 clinical trial evaluating mavorixafor in certain chronic neutropenic disorders may not enable successful completion of the trial(s); the commercial opportunity for mavorixafor in chronic neutropenic disorders may be smaller than anticipated; X4 may be unable to obtain and maintain regulatory approvals; the company may experience uncertainties inherent in the initiation and completion of preclinical studies and clinical trials and clinical development; adverse safety effects might arise from the testing or use of X4 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 ability 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 Annual Report on X4's Form 10-K expected to be filed with the Securities and Exchange Commission (SEC) on March 25, 2025, 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 December 31,		Year Ended December 31,	
	2024	2023	2024	2023
Product revenue, net	$ 1,434	$ —	$ 2,557	$ —
Costs and operating expenses:				
Cost of revenue	302	—	797	—
Research and development	21,702	15,272	81,643	72,017
Selling, general and administrative	15,145	9,927	61,518	35,505
Gain on sale of non-financial asset	—	—	(105,000)	
Total operating expenses	37,149	25,199	38,958	107,522
Loss from operations	(35,715)	(25,199)	(36,401)	(107,522)
Other (expense) income, net	(3,848)	6,102	(739)	6,433
Loss before provision for income taxes	(39,563)	(19,097)	(37,140)	(101,089)
Provision for income taxes	258	33	310	78
Net loss	$ (39,821)	$ (19,130)	$ (37,450)	$ (101,167)
Net loss per share- basic and diluted	$ (0.20)	$ (0.10)	$ (0.19)	$ (0.57)
Weighted average common shares outstanding-basic and diluted	202,933	198,766	201,062	177,812

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

	December 31, 2024	December 31, 2023
Current assets:		
Cash and cash equivalents	$ 55,699	$ 99,216
Accounts receivable	1,070	—
Marketable securities	46,361	15,000
Research and development incentive receivable	640	562
Inventory	2,817	—
Prepaid expenses and other current assets	5,588	7,298
Total current assets	112,175	122,076

Property and equipment, net	776	745
Goodwill	17,351	17,351
Intangible asset, net	10,000	—
Right-of-use assets	4,065	5,650
Other assets	2,080	1,436
Total assets	$ 146,447	$ 147,258
Current liabilities:		
Accounts payable	$ 8,621	$ 8,947
Accrued expenses	23,005	12,816
Current portion of lease liability	1,251	1,099
Total current liabilities	32,877	22,862
Long-term debt, including accretion, net of discount	75,425	54,570
Lease liabilities	1,410	2,612
Other liabilities	14,586	16,115
Total liabilities	124,298	96,159
Total stockholders' equity	22,149	51,099
Total liabilities and stockholders' equity	$ 146,447	$ 147,258

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