



## X4 Pharmaceuticals Reports Third Quarter 2024 Financial Results and Provides Corporate Update

November 13, 2024

*Positive results from completed Phase 2 study of mavorixafor in chronic neutropenia (CN) announced today*

*Pivotal Phase 3 trial of mavorixafor in CN on track to fully enroll in mid-2025*

*U.S. launch of XOLREMDI® (mavorixafor) in WHIM underway; submission of Marketing Authorization Application (MAA) to European Medicines Agency (EMA) expected by early 2025*

*Conference call and webcast today at 8:00 a.m. ET*

BOSTON, Nov. 13, 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 third quarter ended September 30, 2024 and highlighted recent and key upcoming expected milestones.

Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals commented: "Earlier today, we announced results from our completed Phase 2 study of mavorixafor in people with chronic neutropenia that bolster our confidence in delivering a positive outcome from our ongoing pivotal Phase 3 4WARD trial. The data shared today demonstrated that oral, once-daily mavorixafor was well tolerated and meaningfully elevated study participants' blood neutrophil counts. With these new data and strong momentum initiating sites in the 4WARD clinical trial across the globe, we have made significant progress in advancing mavorixafor for the potential treatment of those with chronic neutropenia."

Dr. Ragan added: "Now a few months into the U.S. launch of XOLREMDI (mavorixafor), we are seeing our focus on disease education translate into growing awareness of and screening for WHIM syndrome among likely prescribers, setting a strong foundation for further identifying and treating patients."

### Recent and Key Anticipated Upcoming Milestones

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

- **Positive Phase 2 Clinical Data.** X4 announced this morning positive clinical results from its completed Phase 2 study evaluating mavorixafor in the treatment of people with chronic neutropenia (CN). These study results showed that:
  - Once-daily oral mavorixafor was generally well tolerated,
  - Mavorixafor treatment durably and meaningfully increased participants' mean absolute neutrophil counts (ANC),
  - Physicians were willing and able to reduce injectable granulocyte colony-stimulating factor (G-CSF) therapy in participants also treated with mavorixafor, maintaining mean ANC levels in the normal range, and,
  - In a sub-study assessing neutrophil functionality, the mean percentage of functional circulating neutrophils in representative CN study participants remained comparable to that of healthy donors after 6 Months of mavorixafor dosing.
- **Phase 3 4WARD Trial On Track to Fully Enroll in Mid-2025.** The company is currently enrolling and dosing participants in 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. The company has received protocol regulatory approvals for trial initiation in approximately 85% of targeted countries and approximately 40% of the planned clinical trial sites have been initiated. X4 expects to complete enrollment in the 4WARD trial in mid-2025.

#### **XOLREMDI® (mavorixafor) in WHIM Syndrome**

- **U.S. Launch:** Following the U.S. approval of XOLREMDI (mavorixafor) in April 2024, X4 is now generating revenues from product sales. This quarter, X4 launched a patient-targeted campaign, including a patient-focused [website](#), and a physician peer-to-peer speaker program initially aimed at increasing disease awareness and understanding.
- **Positive Market Research:** To measure the impact of its commercial efforts to date, X4 recently concluded a comprehensive healthcare provider market research study. These results indicate high awareness of WHIM syndrome (>75%), increases in screening for WHIM syndrome, and a growing number of likely prescribers (>80%) considering XOLREMDI for their patients.
- **Industry Conference Engagement:** Since the end of the second quarter, X4 has participated in numerous medical

meetings engaging physicians, nurses, pharmacists, payors, and patient advocacy audiences including: the Consortium of Independent Immunology Clinics (CIIC) Fall Conference, the North American Immuno-Hematology Clinical Education and Research (NICER) Symposium, and the annual meetings of the Immunoglobulin National Society (IgNS), the Academy of Managed Care Pharmacy (AMCP Nexus), the European Society for Immunodeficiencies (ESID), and the International Patient Organisation for Primary Immunodeficiencies (IPOPI).

- **Maximizing the Global Opportunity in WHIM Syndrome:** X4 continues to expect to submit a Marketing Authorization Application (MAA) for the approval of mavorixafor in WHIM syndrome to 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. approval.

### Third Quarter 2024 Financial Results

- **Cash position:** X4 had \$135.8 million in cash, cash equivalents, restricted cash, and short-term marketable securities as of September 30, 2024. The Company believes it has sufficient funds to support 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 September 30, 2024, X4 reported net product revenue of \$0.6 million and cost of revenue of \$0.2 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.
- **Research and Development (R&D) Expenses** were \$19.2 million for the third quarter ended September 30, 2024 as compared to \$19.1 million for the comparable period in 2023. R&D expenses for the third quarter ended September 30, 2024 included \$1.1 million of certain non-cash expenses.
- **Selling, General, and Administrative (SG&A) Expenses** were \$15.7 million for the third quarter ended September 30, 2024 as compared to \$8.1 million for the comparable period in 2023. SG&A expenses for the third quarter ended September 30, 2024 included \$0.7 million of certain non-cash expenses.
- **Net Loss:** X4 reported a net loss of \$36.7 million for the third quarter ended September 30, 2024, as compared to a net loss of \$2.3 million for the comparable period in 2023. Net loss in the current period includes a non-cash loss of \$1.9 million related to the fair value remeasurement of the company's Class C warrant liability. Net loss in the year-ago period included a non-cash gain of \$25.2 million related to the company's Class C warrant liability.

### Conference Call and Webcast

The company will host a conference call and webcast today at 8:00 a.m. ET. 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 and accompanying slide presentation will be accessible through the investor relations section of X4 Pharmaceuticals' website at [www.x4pharma.com](http://www.x4pharma.com). A live Q&A will follow the formal presentation. Following the conclusion of the call and webcast, a replay will be available on the company's 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](http://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](https://clinicaltrials.gov/ct2/show/study/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](http://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, business, plans, or intentions. Forward-looking statements include, without limitation, implied or express statements regarding X4’s expectations as to the success of the commercial launch of XOLREMDI (mavoxifafor), which is approved in the U.S. for use in patients 12 years of age and older with WHIM syndrome (the “Indication”); X4’s belief in its strategy for the commercial launch of XOLREMDI; the potential benefit of XOLREMDI in the Indication; the potential number of patients in the United States with WHIM syndrome and the potential market for XOLREMDI due to unmet potential patient needs; other statements regarding X4’s future operations, financial performance, financial position, prospects, objectives and other future events; the potential therapeutic benefit of mavoxifafor; the initiation, timing, progress, and results of our current and future preclinical studies and clinical trials, including the completed Phase 2 clinical trial in chronic neutropenia and the ongoing Phase 3 4WARD clinical trial and related preparatory work and the period during which the results of the trials will become available, as well as our research and development programs; X4’s use of capital and other financial results, including its financial runway; 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 the risks that: X4’s launch and commercialization efforts in the U.S. with respect to XOLREMDI may not be successful; the number of patients with WHIM syndrome, the unmet need for additional treatment options, and the potential market for XOLREMDI may be significantly smaller than we expect; XOLREMDI may not achieve the clinical benefit, clinical use, or market acceptance we expect or we may encounter reimbursement-related or other market-related issues that impact the success of our commercialization efforts; we may encounter adverse events for XOLREMDI at any stage that negatively impact commercialization; X4 may have difficulty establishing and maintaining an effective sales and marketing organization or suitable third-party alternatives for any approved products; X4 may not be able to obtain regulatory approval for, or successfully commercialize, mavoxifafor or any other product candidate for other chronic neutropenic disorders or any other potential indication; 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; the expected availability, content, and timing of clinical data from X4’s ongoing clinical trials of mavoxifafor may be delayed or unavailable or have unsatisfactory outcomes, including our completed Phase 2 clinical trial and our ongoing Phase 3 clinical trial; the outcomes of preclinical studies or earlier clinical trials will not be predictive of later clinical trial results, including clinical results from our completed Phase 2 clinical trial; the design and rate of enrollment for clinical trials, including the current design of a Phase 3 clinical trial evaluating mavoxifafor in certain chronic neutropenic disorders may not enable successful completion of the trial(s); the commercial opportunity for XOLREMDI in WHIM syndrome and other chronic neutropenic disorders may be smaller than we anticipate and X4’s potential future revenue from XOLREMDI may be adversely affected; X4 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; initial or interim results from a clinical trial may not be predictive of the final results of the trial or the results of future trials, including assessing the ability of mavoxifafor monotherapy to durably increase absolute neutrophil count in patients with chronic neutropenic; adverse safety effects arise from the testing or use of our product and product candidates; general macroeconomic and geopolitical conditions which could impact X4’s business; X4 may be unable to raise additional capital; there is substantial doubt about X4’s ability to continue as a going concern; there will be changes in expected or existing competition; there will be changes in the regulatory environment; unexpected litigation or other disputes; the need to align with our collaborators may hamper or delay our development and commercialization efforts or increase our costs; 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 Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on August 8, 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		Nine Months Ended	
	September 30,		September 30,	
	2024	2023	2024	2023
Product revenue, net	\$ 560	\$ —	\$ 1,123	\$ —
Costs and operating expenses:				
Cost of revenue	227	—	495	—
Research and development	19,173	19,081	59,941	56,745
Selling, general and administrative	15,660	8,133	46,373	25,578
Gain on sale of non-financial asset	—	—	(105,000)	—
Total operating expense	35,060	27,214	1,809	82,323
Loss from operations	(34,500)	(27,214)	(686)	(82,323)
Other (expense) income, net:	(2,181)	24,935	3,109	331
(Loss) income before provision for income taxes	(36,681)	(2,279)	2,423	(81,992)
Provision for income taxes	15	26	52	45
Net (loss) income	\$ (36,696)	\$ (2,305)	\$ 2,371	\$ (82,037)

Net (loss) income per share: basic	\$ (0.18)	\$ (0.01)	\$ 0.01	\$ (0.48)
Weighted average shares--basic	200,865	196,988	200,434	170,751
Net (loss) income per share: diluted	\$ (0.18)	\$ (0.01)	\$ 0.01	\$ (0.48)
Weighted average shares--diluted	200,865	196,988	200,611	170,751

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

	<b>September 30, 2024</b>	<b>December 31, 2023</b>
<b>Current assets:</b>		
Cash and cash equivalents	\$ 97,412	\$ 99,216
Marketable securities	37,565	15,000
Research and development incentive receivable	1,073	562
Inventory	2,445	—
Prepaid expenses and other current assets	4,477	7,298
<b>Total current assets</b>	<b>142,972</b>	<b>122,076</b>
Property and equipment, net	820	745
Goodwill	17,351	17,351
Intangible asset, net	10,188	—
Right-of-use assets	4,471	5,650
Other assets	2,363	1,436
<b>Total assets</b>	<b>\$ 178,165</b>	<b>\$ 147,258</b>
<b>Current liabilities:</b>		
Accounts payable	\$ 8,577	\$ 8,947
Accrued expenses	19,459	12,816
Current portion of lease liability	1,222	1,099
<b>Total current liabilities</b>	<b>29,258</b>	<b>22,862</b>
Long-term debt, including accretion, net of discount	75,224	54,570
Lease liabilities	1,757	2,612
Warrant liability	11,087	15,683
Other liabilities	1,218	432
<b>Total liabilities</b>	<b>118,544</b>	<b>96,159</b>
Total stockholders' equity	59,621	51,099
<b>Total liabilities and stockholders' equity</b>	<b>\$ 178,165</b>	<b>\$ 147,258</b>

**Company Contact:**

José Juves  
Head of Corporate & Patient Affairs  
[jose.juves@x4pharma.com](mailto:jose.juves@x4pharma.com)

**Investor Contact:**

Daniel Ferry  
Managing Director, LifeSci Advisors  
[daniel@lifesciadvisors.com](mailto:daniel@lifesciadvisors.com)  
(617) 430-7576

