X4 Pharmaceuticals Reports First Quarter 2026 Financial Results and Provides Corporate Update

May 6, 2026

- Global 4WARD Phase 3 Chronic Neutropenia Trial On Track to Complete Enrollment by the end of Q3 2026 -

- European Commission Approval of XOLREMDI® (Mavorixafor) Provides the First and Only Authorized Treatment for Patients with WHIM Syndrome in the European Union -

- Balance Sheet Provides Cash Runway through 2028 -

BOSTON, May 06, 2026 (GLOBE NEWSWIRE) -- **X4 Pharmaceuticals** (Nasdaq: XFOR), a company focused on improving the lives of people with rare hematology diseases, today reported financial results for the first quarter ended March 31, 2026 and provided a corporate update.

“Our top priority remains the execution and full enrollment of the pivotal 4WARD Phase 3 trial of mavorixafor in chronic neutropenia,” said Adam Craig, M.D., Ph.D., Executive Chairman of X4 Pharmaceuticals. “We remain on track to complete enrollment by the end of the third quarter of this year. In addition, we are pleased that the European Commission granted marketing authorization for XOLREMDI® (mavorixafor) for the treatment of patients with WHIM syndrome in the European Union, reinforcing the broader potential of our program to advance therapies for individuals affected by rare hematologic disorders. We look forward to a smooth transfer of the marketing authorization application to our European partner, Norgine, later this year as they proceed with commercialization plans throughout Europe.”

Recent Accomplishments and Updates

- Continued to advance enrollment in the global 4WARD Phase 3 trial of oral, once-daily mavorixafor (with or without G-CSF) in patients with congenital, acquired primary autoimmune, or idiopathic chronic neutropenia, supported by several targeted initiatives:
  - Expanded to over 110 active clinical trial sites worldwide, including more than 20 in the U.S., enhancing enrollment and regulatory alignment;
  - Increased global Medical Affairs engagement including the implementation of a dedicated patient referral pathway for physicians; and,
  - Leveraging data-driven approaches including database mining to identify potential participants.
- The European Commission granted marketing authorization for XOLREMDI® (mavorixafor) for the treatment of patients with WHIM syndrome in the European Union (EU). The approval follows a positive opinion from the European Medicines Agency’s Committee for Medicinal Products for Human Use (CHMP). European commercialization will be led by X4 Pharmaceuticals’ partner, Norgine. Under the terms of the license and supply agreement, X4 will receive up to €226 million contingent upon the achievement of certain regulatory and commercial milestones, in addition to escalating double-digit royalties of up to the mid-twenties on any future net sales in the licensed territories.

First Quarter Financial Results

- **Cash Position:** As of March 31, 2026, the Company’s cash position was $233.7 million.
- **Revenue:** Revenue was $2.7 million and $28.8 million for the three months ended March 31, 2026 and 2025, respectively. Net product sales in both periods were primarily attributable to XOLREMDI product sales in the United States. Revenue for the three months ended March 31, 2025 included $27.9 million of license revenue attributable to the Norgine license and supply agreement.
- **Research and Development (R&D) expenses:** R&D Expenses were $15.5 million for the three months ended March 31, 2026 as compared to $18.5 million for the three months ended March 31, 2025. The decrease is primarily attributable to decreased headcount following the 2025 strategic restructuring.
- **General and Administrative (G&A) expenses**: G&A Expenses were $7.0 million for the three months ended March 31, 2026 as compared to $15.0 million for the three months ended March 31, 2025. The decrease is primarily attributable to decreased headcount following the 2025 strategic restructuring.
- **Net (Loss) Income:** Net loss for the three months ended March 31, 2026 was $(20.2) million, or $(0.16) per share, as compared to net income for the three months ended March 31, 2025 of $0.3 million or $0.04 per share. The difference was due to the Norgine license revenue recognized in the first quarter of 2025, partially offset by a significant reduction in

operating expenses.

About Chronic Neutropenia and Mavorixafor

Chronic neutropenia is a primary, rare blood condition characterized by abnormally low levels of circulating neutrophils in the blood lasting more than three months, persistently or intermittently. As a result, people with chronic neutropenia are at an increased risk of serious and life-threatening infections and reduced quality of life. Neutrophils are retained in the bone marrow by the CXCR4/CXCL12 axis, creating a reserve of cells. Mavorixafor is a small molecule delivered in a capsule for oral dosing as a selective antagonist of the chemokine receptor, CXCR4. Down-regulation of the CXCR4 receptor by mavorixafor has been shown to mobilize functional neutrophils from the bone marrow into the peripheral bloodstream across multiple disease states. The level of circulating neutrophils is typically determined by the absolute neutrophil count (ANC) obtained from a blood draw.

About the 4WARD Clinical Trial

The 4WARD trial is a global, pivotal Phase 3 clinical trial evaluating the efficacy, safety, and tolerability of oral, once-daily mavorixafor (with or without G-CSF) in patients 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 176 patients aged 12 years and older with confirmed trough absolute neutrophil count (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 endpoints of the study are the reduction in annualized infection rate and positive ANC response. For more information, visit clinicaltrials.gov (NCT06056297).

About X4 Pharmaceuticals

X4 Pharmaceuticals is a company focused on improving the lives of people with rare hematology diseases by developing and commercializing innovative therapies in areas with significant unmet needs. Leveraging expertise in diseases of the immune system and CXCR4 biology, X4 has successfully developed mavorixafor, an orally available CXCR4 antagonist that is commercially available in the U.S. as XOLREMDI® in its first indication. The Company is currently conducting a global, pivotal Phase 3 clinical trial (4WARD) evaluating mavorixafor in chronic neutropenic disorders. The U.S. FDA has granted Fast Track designation to mavorixafor for the treatment of chronic neutropenia. 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 Company’s ability to obtain and maintain regulatory approval for its product candidates, plans for the commercialization of XOLREMDI in the European Union by Norgine, the potential achievement of milestones and receipt of royalties under the Company’s licensing and supply agreement with Norgine, the expected design and enrollment of the Company’s clinical trials, including expected timing for full enrollment in 4WARD, the sufficiency of the Company’s cash resources and its expected cash runway, and future plans for the Company. 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 even if approved, mavorixafor may not ultimately be commercially successful; the Company is unable to initiate and complete its clinical trials, including the 4WARD trial; and other risks and uncertainties, including those described in the section entitled “Risk Factors” in X4’s most recent Annual Report on Form 10-K, as well as in other filings X4 makes with the Securities and Exchange Commission, including its Quarterly Reports 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.

X4 Investor Contact:
Remy Bernarda
Jenny Kobin
IR Advisory Solutions
IR@X4pharma.com

Source: X4 Pharmaceuticals, Inc.

(Tables Follow)

X4 PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended March 31,	
	2026	**2025**
License and other revenue	$ 239	$ 27,865
Product revenue, net	2,469	942
Total revenue	2,708	28,807
Costs and operating expenses:		
Cost of revenue	598	4,716
Research and development	15,450	18,513
General and administrative	6,966	15,021

Total operating expense	23,014	38,250
Loss from operations	(20,306)	(9,443)
Other income, net	65	9,759
(Loss) income before provision for income taxes	(20,241)	316
Provision for income taxes	—	34
Net (loss) income	$ (20,241)	$ 282
Net (loss) income per share: basic	$ (0.16)	$ 0.04
Weighted average shares outstanding: basic	126,289	6,840
Net (loss) income per share: diluted	$ (0.16)	$ 0.04
Weighted average shares outstanding: diluted	126,289	6,869

Balance Sheet Data (unaudited)

	(amounts in thousands)	
	March 31, 2026	**December 31, 2025**
Cash and cash equivalents	$ 216,908	$ 217,049
Accounts receivable	1,113	573
Marketable securities	16,812	35,949
Working capital	218,211	235,820
Total assets	270,281	290,461
Current portion of long-term debt	—	—
Total stockholders’ equity	168,450	186,290