X4 Pharmaceuticals Reports Fourth Quarter and Full Year 2025 Financial Results and Provides Corporate Update

March 17, 2026

- Global 4WARD Phase 3 Chronic Neutropenia Trial On Track to Complete Enrollment in Q3 2026 -

- Positive Opinion from European Medicines Agency Recommending Marketing Authorization of Mavorixafor for the Treatment of WHIM Syndrome in the European Union -

- Balance Sheet Provides Cash Runway through 2028 -

BOSTON, March 17, 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 fourth quarter and year ended December 31, 2025 and provided a corporate update.

“Our focus remains on our pivotal 4WARD trial in chronic neutropenia as we execute our strategy to deliver life-changing therapies to people living with rare blood diseases,” said Adam Craig, M.D., Ph.D., Executive Chairman of X4 Pharmaceuticals. “We have sharpened our operational focus and implemented targeted initiatives to drive continued progress in 4WARD with enrollment on track for completion in the third quarter of this year. Additionally, we were pleased to receive the positive opinion from the European Medicines Agency recommending the potential European approval of mavorixafor in WHIM syndrome, which would be the first and only therapy in Europe to treat this devastating condition.”

Recent Accomplishments and Updates

- Deployed a series of proactive measures to increase 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, including:
  - Expanding the number of current active clinical trial sites to over 100, including approximately 20 in the U.S.;
  - Enhancing our global Medical Affairs activities to increase Medical Science Liaison (MSL) field engagement and site interaction to educate physicians on our 4WARD trial and mavorixafor’s potential in treating chronic neutropenia;
  - Establishing a patient referral pathway for physicians to assist them in finding trial sites for their chronic neutropenia patients; and
  - Investing in database mining to identify potential participants.
- European Medicines Agency’s (EMA) Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion recommending marketing authorization of mavorixafor the treatment of WHIM (warts, hypogammaglobulinemia, infections, and myelokathexis) syndrome in the European Union (EU). The positive opinion will now be reviewed by the European Commission (EC) with a final approval decision anticipated in the second quarter of 2026.
- Cash runway through 2028 due to recent equity financings and operating expense reductions.

Fourth Quarter and Full Year Financial Results

- Net product sales of $2.3 million and $6.5 million for the three and twelve months ended December 31, 2025, respectively, were entirely attributable to XOLREMDI product sales in the United States.
- License and other revenue of $0.3 million and $28.6 million for the three and twelve months ended December 31, 2025, respectively, were entirely attributable to the Company's Norgine out-licensing agreement.
- Operating loss was $23.7 million and $86.9 million for the three and twelve months ended December 31, 2025, respectively.
- Net loss for the three and twelve months ended December 31, 2025 was $23.9 million, or $(0.22) per share, and $79.2 million, or $(1.87) per share, respectively.
- Cash, cash equivalents and short-term investments totaled $253.0 million as of December 31, 2025, which management believes will enable the Company to fund its operations through 2028.

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, including expected timing of the EC’s approval decision regarding mavorixafor for the treatment of WHIM in the EU, 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: the Company is unable to obtain or maintain regulatory approval for its product candidates, including for mavorixafor for the treatment of WHIM in the European Union; 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 X4’s 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 December 31,		Year Ended December 31,	
	2025	2024	2025	2024
License and other revenue	$ 297	$ 1,434	$ 28,590	$ —
Product revenue, net	2,271	—	6,523	2,557
Total revenue	2,568	1,434	35,113	2,557
Costs and operating expenses:				
Cost of revenue	364	302	5,757	797
Research and development	18,521	21,702	72,723	81,643
Selling, general and administrative	7,382	15,145	43,516	61,518
Gain on sale of non-financial asset		—	—	(105,000)
Total operating expenses	26,267	37,149	121,996	38,958
Loss from operations	(23,699)	(35,715)	(86,883)	(36,401)
Other (expense) income, net	(226)	(3,848)	7,725	(739)
Loss before provision for income taxes	(23,925)	(39,563)	(79,158)	(37,140)

Provision for income taxes	—	258	41	310
Net loss	$ (23,925)	$ (39,821)	$ (79,199)	$ (37,450)
Net loss per share- basic and diluted	$ (0.22)	$ (5.89)	$ (1.87)	$ (5.59)
Weighted average shares outstanding-basic and diluted	110,556	6,764	42,293	6,702

Balance Sheet Data (unaudited)

	(amounts in thousands)	
	December 31, 2025	**December 31, 2024**
Cash and cash equivalents	$ 217,049	$ 55,699
Accounts receivable	573	1,070
Marketable securities	35,949	46,361
Working capital	235,820	79,298
Total assets	290,461	146,447
Current portion of long-term debt	—	—
Total stockholders' equity	186,290	22,149