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

August 6, 2026

- Strengthened Clinical Operations to Continue Progress in Enrolling Global 4WARD Phase 3 Chronic Neutropenia Trial -

- 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 into 2029 -

BOSTON, Aug. 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 second quarter ended June 30, 2026 and provided a corporate update.

“Since we began leading X4 last August, we have taken meaningful actions to strengthen the execution of the mavorixafor Phase 3 4WARD trial in chronic neutropenia, positioning 4WARD for the successful completion of enrollment,” said Adam Craig, M.D., Ph.D., Executive Chairman of X4 Pharmaceuticals. “Following our complete overhaul of the clinical operating infrastructure and engagement of a top oncology Clinical Research Organization (CRO) to manage the Phase 3 trial, we are now in a position to revisit the sample size of the study with the FDA. We expect to provide an update on our meeting with the FDA and the completion of enrollment by the end of the third quarter.”

“Alongside these operational improvements, we continue to actively engage with the medical community to educate physicians on the potential benefits of mavorixafor as an improved treatment option for patients, including a strong presence at this year’s EHA Congress. During the quarter, we also achieved an important milestone with the European Commission’s marketing authorization of XOLREMDI® for WHIM syndrome, supporting the therapeutic potential of mavorixafor across rare hematologic conditions,” concluded, Dr. Craig.

Recent Accomplishments and Updates

- Transitioned management of the Phase 3 4WARD trial to a premier Clinical Research Organization (CRO) to further strengthen the study execution and support completion of enrollment.
- Advanced 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 including activation of over 110 active clinical trial sites worldwide, ongoing Medical Affairs engagement, and utilizing data-driven approaches to identify potential participants.
- Continued to expand awareness in the medical community with a strong presence at the European Hematology Association (EHA) Congress including a publication entitled, “A Pivotal Phase 3 Study To Investigate Efficacy, Safety, and Tolerability of Mavorixafor in Participants with Primary Chronic Neutropenia”. The Medical Affairs team also hosted a booth and held an investigator meeting to engage with physicians regarding the 4WARD trial and the potential benefits of mavorixafor in chronic neutropenia.
- Received marketing authorization for XOLREMDI® (mavorixafor) by the European Commission 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. In July 2026, the EU marketing authorization was transferred to Norgine. Under the terms of the license and supply agreement, X4 could receive up to an additional €221 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 June 30, 2026, the Company’s cash position was $208.0 million.
- **Revenue:** Revenue was $8.8 million and $11.5 million for the three and six months ended June 30, 2026, respectively. Revenue included net product sales of $2.4 million and $4.8 million for the three and six months ended June 30, 2026, respectively, attributable to XOLREMDI® product sales in the United States. License and other revenue for the three and

six month period ended June 30, 2026 included $6.4 million and $6.7 million, respectively, attributable to the Company's Norgine license and supply agreement. Revenue was $2.0 million and $30.8 million for the three and six months ended June 30, 2025, respectively. Revenue for the six months ended June 30, 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 $15.1 million and $30.5 million for the three and six months ended June 30, 2026 as compared to $18.4 million and $36.9 million for the same periods in prior year. The decrease in both periods is primarily attributable to decreased headcount following the 2025 strategic restructuring, partially offset by higher clinical costs, primarily CRO costs related to our 4WARD trial.
- **General and Administrative (G&A) expenses:** G&A expenses were $8.5 million and $15.5 million for the three and six months ended June 30, 2026 as compared to $9.5 million and $24.6 million for the same periods in the prior year. The decrease in both periods is primarily attributable to a reduction in sales and marketing expenses, a reduction in outside legal expenses, and reductions in compensation costs due to lower head count in general and administrative functions.
- **Net Loss:** Net loss for the three months ended June 30, 2026 was $16.2 million, or $(0.13) per share, as compared to net loss for the three months ended June 30, 2025 of $25.7 million, or $(3.47) per share. Net loss for the six months ended June 30, 2026 was $36.4 million, or $(0.29) per share, as compared to net loss of $25.5 million, or $(3.59) per share, for the same period in 2025.

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.

Source: X4 Pharmaceuticals, Inc.

(Tables Follow)

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

(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	**2025**	**2026**	**2025**
License and other revenue	$ 6,433	$ 229	$ 6,672	$ 28,094
Product revenue, net	2,373	1,744	4,842	2,686
Total revenue	8,806	1,973	11,514	30,780
Costs and operating expenses:				
Cost of revenue	1,617	326	2,215	5,042
Research and development	15,079	18,352	30,529	36,865
General and administrative	8,501	9,527	15,467	24,548
Total operating expenses	25,197	28,205	48,211	66,455
Loss from operations	(16,391)	(26,232)	(36,697)	(35,675)
Other income, net	236	528	301	10,287
Loss before provision for income taxes	(16,155)	(25,704)	(36,396)	(25,388)
Provision for income taxes	2	37	2	71
Net loss	$ (16,157)	$ (25,741)	$ (36,398)	$ (25,459)
Net loss per share- basic and diluted	$ (0.13)	$ (3.47)	$ (0.29)	$ (3.59)
Weighted average shares outstanding-basic and diluted	126,344	7,413	126,317	7,096

Balance Sheet Data (unaudited)

	(amounts in thousands)	
	June 30, 2026	**December 31, 2025**
Cash and cash equivalents	$ 195,095	$ 217,049
Accounts receivable	2,047	573
Marketable securities	12,874	35,949
Working capital	199,489	235,820
Total assets	253,063	290,461
Current portion of long-term debt	—	—
Total stockholders' equity	155,454	186,290

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