X4 Pharmaceuticals Reports Third Quarter 2025 Financial Results and Provides Corporate Update

November 5, 2025

Company's strategic focus and highest priority is now the advancement of the 4WARD Phase 3 chronic neutropenia trial
Previously announced workforce reductions resulted in $13M annualized cost savings
Successful completion of two financial transactions totaling $240.3M; cash runway now extends to the end of 2028

BOSTON, Nov. 05, 2025 (GLOBE NEWSWIRE) -- **X4 Pharmaceuticals** (Nasdaq: XFOR), a company driven to improve the lives of people with rare hematology diseases, today reported financial results for the third quarter ended September 30, 2025 and provided a corporate update.

"The third quarter of 2025 was a time of corporate restructuring at X4 with the start of a new leadership team and a renewed focus on chronic neutropenia," said Adam Craig, M.D., Ph.D., Executive Chairman of X4 Pharmaceuticals. "With a strengthened financial position through two successful financings totaling $240.3 million, our primary focus is now on the completion of the 4WARD Phase 3 pivotal trial of mavorixafor in patients with moderate and severe chronic neutropenia, which has a potential addressable market of 15,000 patients in the US. With a cash runway to the end of 2028, we are now positioned to unlock mavorixafor's full potential and to establish X4 as a world-class rare hematology company."

Recent Accomplishments and Updates

- Since early August, the Company has initiated a number of measures to restructure its operations:
  - A shift in the primary focus of the Company to the successful completion of the 4WARD Phase 3 pivotal trial of mavorixafor in patients with moderate and severe chronic neutropenia.
  - A deprioritization of the commercialization of mavorixafor (XOLREMDI) for patients with WHIM syndrome, while maintaining patient access.
  - A 50% reduction in the workforce (expected to generate approximately $13 million in annualized cost savings) with continued cost cutting measures.
  - An increase in the enrollment target for the pivotal Phase 3 4WARD study to 176 patients with enrollment now expected to be completed in third quarter of 2026.
  - The promotion of John Volpone to the role of Chief Operating Officer, in addition to his responsibilities as President.
  - Dr. Adam Craig expanded his role to include oversight of clinical development activities.
- Since August, the Company raised $240.3 million in gross proceeds from two successful financings: the closing of a $155.3 million underwritten public offering and an $85 million upsized private placement.
- With a strengthened cash runway to the end of 2028, X4 is now expected to be able to complete the 4WARD trial, file a potential sNDA for the chronic neutropenia indication, and, if successful, launch mavorixafor in this new indication by the end of 2028.

Third-Quarter and Recent 2025 Financial Results
Net product sales of $1.6 million and $4.3 million for the three and nine months ended September 30, 2025, respectively, were entirely attributable to XOLREMDI product sales in the United States. Net product sales were $0.6 million and $1.1 million for the three and nine months ended September 30, 2024, respectively. License and other revenue of $0.2 million and $28.3 million for the three and nine months ended September 30, 2025, respectively, were entirely attributable to the Company's Norgine out-licensing agreement. Operating loss was $27.5 million and $34.5 million for the three months ended September 30, 2025, and 2024, respectively, and $63.2 million and $0.7 million for the nine months ended September 30, 2025, and 2024, respectively. The decrease in operating loss between the three-month periods ended September 30, 2025, and 2024 was primarily attributable to the impact of our 2025 Strategic Restructuring activities. Operating expenses for the nine months ended September 30, 2024, are net of a gain of $105.0 million realized on the sale of a priority review voucher. Exclusive of this gain, the decrease in operating loss between the nine-month periods ended September 30, 2025, and 2024 was primarily attributable to the impact of the Company's strategic restructuring activities undertaken in 2025.

Net loss for the three months ended September 30, 2025 was $29.8 million, or $0.69 for basic and diluted loss per share, compared to net loss of $36.7 million, or $5.48 for basic and diluted loss per share, for the same period in 2024. Net loss for the nine months ended September 30, 2025 was $55.3 million, or $2.87 for basic and diluted loss per share, compared to net income of $2.4 million, or $0.35 for basic and diluted income per share, for the same period in 2024.

Cash, cash equivalents and short-term investments totaled $122.2 million for the period ended September 30, 2025. On October 27, 2025, the Company completed a public offering with net proceeds of $145.6 million, which management believes will enable the Company to fund its operations into the end of 2028.

About Chronic Neutropenia and Mavorixafor

Chronic neutropenia is a primary, rare blood condition lasting more than three months, persistently or intermittently, and characterized by low levels of circulating neutrophils and increased risk of serious and life-threatening 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 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 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 endpoints of the study are the reduction in 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 hematology diseases and significant unmet needs. Leveraging expertise in CXCR4, 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 patients 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 expected cost savings from the workforce reduction, the expected results of the restructuring of business operations, the sufficiency of the Company’s cash resources and its expected cash runway, the potential addressable market for moderate and severe chronic neutropenia, the timing for launch of mavorixafor 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 workforce reduction does not result in the anticipated cost savings described herein; the results of the restructuring of business operations are not as anticipated; 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 report 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.

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(Tables Follow)

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
License and other revenue	$ 199	$ —	$ 28,293	$ —
Product revenue, net	1,566	560	4,252	1,123
Total revenue	1,765	560	32,545	1,123
Costs and operating expenses:				
Cost of revenue	351	227	5,393	495
Research and development	17,337	19,173	54,202	59,941
Selling, general and administrative	11,586	15,660	36,134	46,373
Gain on sale of non-financial asset	—	—	—	(105,000)
Total operating expenses	29,274	35,060	95,729	1,809
Loss from operations	(27,509)	(34,500)	(63,184)	(686)
Other income, net	(2,336)	(2,181)	7,951	3,109
(Loss) income before provision for income taxes	(29,845)	(36,681)	(55,233)	2,423
Provision for income taxes	(30)	15	41	52
Net (loss) income	$ (29,815)	$ (36,696)	$ (55,274)	$ 2,371
Net (loss) income per share- basic.	$ (0.69)	$ (5.48)	$ (2.87)	$ 0.35
Weighted average shares outstanding-basic	43,273	6,696	19,243	6,681

Net (loss) income per share- diluted	$ (0.69)	$ (5.48)	$ (2.87)	$ 0.35
Weighted average shares outstanding-diluted	43,273	6,696	19,243	6,687

Balance Sheet Data (unaudited)

	(amounts in thousands)	
	September 30, 2025	**December 31, 2024**
Cash and cash equivalents	$ 69,632	$ 55,699
Accounts receivable	891	1,070
Marketable securities	52,562	46,361
Working capital	109,299	79,298
Total assets	163,555	146,447
Current portion of long-term debt	—	—
Total stockholders' equity	61,619	22,149