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

November 4, 2026

- 4WARD Phase 3 Chronic Neutropenia Trial Expected to Achieve Target Enrollment in Q4 2026 -

- Initiating 4STEPS Phase 2b Trial Evaluating G-CSF Dose Reduction with Mavorixafor; Enrollment of First Participant Expected in Q1 2027 -

- Investor Day Highlights Significant Market Opportunity for Mavorixafor in Primary Chronic Neutropenia -

- Balance Sheet Provides Cash Runway into Q1 2029 -

BOSTON, Nov. 04, 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 third quarter ended September 30, 2026 and provided a corporate update.

"With topline data from both our global 4WARD trial and our new 4STEPS trial expected in the first quarter of 2028, we believe mavorixafor is uniquely positioned to transform the treatment landscape for patients with primary chronic neutropenia," said Adam Craig, M.D., Ph.D., Executive Chairman of X4 Pharmaceuticals. "We have made significant progress enrolling our 4WARD trial and remain on track to complete our target enrollment of 126 participants by the end of this year. At our recent, well-received Investor Day, we outlined the design of our Phase 2b 4STEPS trial designed to evaluate how to safely reduce G-CSF dosage after starting mavorixafor while maintaining stable neutrophil counts. We also shared new market research supporting the significant commercial opportunity in this disease targeting 15,000 to 30,000 patients in the United States, subject to FDA labeling."

Recent Accomplishments and Updates

- Positive outcome from a Type C meeting with the U.S. Food and Drug Administration (FDA), which led to the optimized 4WARD trial size while maintaining adequate overall power for the trial's co-primary endpoints of absolute neutrophil count (93%) and annualized infection rate (89%) for the intent-to-treat population.
- Advancement of enrollment in the global 4WARD trial of mavorixafor in primary chronic neutropenia, with enrollment of 126 participants expected to complete in the fourth quarter of 2026 and topline data expected in the first quarter of 2028, supporting a potential FDA approval in late 2028 or early 2029.
- Introduced 4STEPS, a planned Phase 2b trial in approximately 25 patients designed to evaluate whether G-CSF dosage can be safely reduced after starting mavorixafor while maintaining stable neutrophil counts, with the first participant expected to be enrolled in the first quarter of 2027 and topline data expected in the first quarter of 2028.
- Hosted virtual Investor Day presenting market research that identified approximately 15,000 U.S. patients mirroring the 4WARD trial population, with potential upside to approximately 30,000 patients, subject to FDA labeling.
- Secured a senior term loan facility of up to $150 million with K2 HealthVentures LLC, replacing a prior loan agreement and strengthening the Company's financial flexibility.

Third Quarter and Recent Financial Results

- **Cash Position:** As of September 30, 2026, the Company's cash, cash equivalents and marketable securities were $187.7 million.
- **Revenue:** Revenue was $2.9 million and $14.4 million for the three and nine months ended September 30, 2026, respectively. Revenue included net product sales of $2.8 million and $7.6 million for the three and nine months ended September 30, 2026, respectively, attributable to XOLREMDI® product sales in the United States. License and other revenue for the three and nine months ended September 30, 2026 included $0.1 million and $6.8 million, respectively, attributable to our Norgine license and supply agreement. Revenue was $1.8 million and $32.5 million for the three and nine months ended September 30, 2025, respectively.
- **Research and Development (R&D) expenses:** R&D expenses were $13.3 million and $43.9 million for the three and nine months ended September 30, 2026 as compared to $17.3 million and $54.2 million for the same periods in the prior year. The decrease in both periods is primarily attributable to decreased headcount from the 2025 strategic restructuring. For the nine-month period, the decrease was partially offset by higher costs for the 4WARD trial.
- **General and Administrative (G&A) expenses**: G&A expenses were $9.0 million and $24.5 million for the three and nine months ended September 30, 2026 as compared to $11.6 million and $36.1 million for the same periods in the prior year.

The decrease in both periods is primarily attributable to reductions in outside legal expenses, compensation costs due to lower headcount in general and administrative functions. The decrease for the nine-month period was also due to a reduction in sales and marketing expenses related to XOLREMDI.

- **Net Loss:** Net loss for the three months ended September 30, 2026 was $20.2 million, or $(0.16) per share, as compared to net loss for the three months ended September 30, 2025 of $29.8 million, or $(0.69) per share. Net loss for the nine months ended September 30, 2026 was $56.6 million, or $(0.45) per share, as compared to net loss of $55.3 million, or $(2.87) per share, for the same period in 2025.

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 medical needs. Leveraging expertise in diseases of the immune system and CXCR4 biology, X4 has successfully developed mavorixafor, an orally available CXCR4 antagonist that is approved in the U.S. and EU as XOLREMDI® for patients with WHIM syndrome. The Company is currently conducting a global, pivotal Phase 3 clinical trial (4WARD) evaluating mavorixafor in primary chronic neutropenia. 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 expected timing of topline data from the 4WARD and 4STEPS trials, the design and expected timing of initiation and enrollment of the 4STEPS trial, the Company’s plans to submit additional information to the FDA regarding the power of the 4WARD trial for its key secondary endpoints, the potential timing of an sNDA submission for and FDA approval of mavorixafor in PCN, the estimated size of the U.S. patient population and market opportunity for mavorixafor in PCN, the availability of additional term loans under the Company’s loan agreement with K2 HealthVentures LLC, 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 and 4STEPS trials, on the expected timelines or at all; the FDA may not agree that the reduced sample size of the 4WARD trial provides adequate power for its key secondary endpoints, or that the safety database is adequate to support an sNDA; topline data from the 4WARD trial may not be positive or sufficient to support approval; the Company’s market research may not accurately reflect the size of the addressable patient population or the scope of any approved label; the Company may not be able to comply with the covenants under its loan agreement with K2 HealthVentures LLC, and additional term loans under that agreement are subject to the lenders’ discretionary approval; the Company will need to raise additional capital to fund its operations, which may not be available on acceptable terms or at all; 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.

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Source: X4 Pharmaceuticals, Inc.

(Tables Follow)

X4 PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(In thousands, except per share amounts)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
License and other revenue	$ 106	$ 199	$ 6,778	$ 28,293
Product revenue, net	2,773	1,566	7,615	4,252
Total revenue	2,879	1,765	14,393	32,545
Costs and operating expenses:				
Cost of revenue	513	351	2,728	5,393
Research and development	13,343	17,337	43,872	54,202
General and administrative	9,014	11,586	24,481	36,134
Total operating expenses	22,870	29,274	71,081	95,729

Loss from operations	(19,991)	(27,509)	(56,688)	(63,184)
Other (expense) income, net	(204)	(2,336)	97	7,951
Loss before provision for income taxes	(20,195)	(29,845)	(56,591)	(55,233)
Provision for income taxes	3	(30)	5	41
Net loss	$ (20,198)	$ (29,815)	$ (56,596)	$ (55,274)
Net loss per share: basic and diluted	$ (0.16)	$ (0.69)	$ (0.45)	$ (2.87)
Weighted average shares outstanding: basic and diluted	126,524	43,273	126,326	19,243

Balance Sheet Data (unaudited)

	(amounts in thousands)	
	September 30, 2026	**December 31, 2025**
Cash and cash equivalents	$ 185,801	$ 217,049
Accounts receivable	1,470	573
Marketable securities	1,900	35,949
Working capital	183,150	235,820
Total assets	231,816	290,461
Current portion of long-term debt	—	—
Total stockholders' equity	138,883	186,290