X4 Pharmaceuticals Announces Positive Outcome from FDA Type C Meeting

September 8, 2026

- FDA Agrees to Reduced Sample Size for the Global 4WARD Phase 3 Chronic Neutropenia Trial -

- Expect Completion of Enrollment by Year-End 2026 and Topline Data in H1 2028 -

BOSTON, Sept. 08, 2026 (GLOBE NEWSWIRE) -- **X4 Pharmaceuticals** (Nasdaq: XFOR), a company focused on improving the lives of people with rare hematology diseases, today announced the positive outcome of its Type C Meeting with the U.S. Food and Drug Administration (FDA) related to the global 4WARD Phase 3 clinical trial. The purpose of the meeting was to gain alignment on a revised sample size and associated statistical power and the size of the safety database to support a supplemental new drug application (sNDA) for mavorixafor for the treatment of chronic neutropenia in 4WARD.

The FDA agreed with the Company's analysis that the reduction in sample size for the 4WARD trial from 176 to 126 participants maintains adequate overall power for the co-primary endpoints of the reduction in annualized infection rate and positive absolute neutrophil count (ANC) response. In addition, the FDA indicated that the proposed safety database would be acceptable to support a potential sNDA.

"After a rigorous re-examination of the statistical plan for 4WARD, we determined that the trial was overpowered and that we could reduce the sample size to 126 participants while preserving good statistical power for our co-primary endpoints," said Adam Craig, M.D., Ph.D., Executive Chairman of X4 Pharmaceuticals. "We are pleased that the FDA agreed with our analysis. With continued execution from our new Clinical Research Organization (CRO), we expect to complete the enrollment of 4WARD by the end of this year and report topline data in the first half of 2028."

"With an estimated 15,000 patients experiencing serious and/or recurring infections as a result of their chronic neutropenia, we continue to believe mavorixafor can address a significant market opportunity based on its unique value proposition for the treatment of this rare disease with very limited therapy options," concluded Dr. Craig.

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 126 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 co-primary endpoints of the study are the reduction in annualized infection rate and positive absolute neutrophil count (ANC) response. For more information, visit clinicaltrials.gov (NCT06056297).

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 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; the FDA's feedback provided in connection with the Type C meeting is not binding on the FDA and the FDA may take a different position in connection with its review of any sNDA; the reduced sample size may adversely affect the ability of the 4WARD trial to demonstrate statistically significant results with respect to its co-primary endpoints; enrollment in the 4WARD trial may not be completed within the timeframe described in this press release, 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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