



X4 Pharmaceuticals Highlights Clinical Progress and Market Opportunity for Mavorixafor in Primary Chronic Neutropenia During Virtual Investor Day

October 29, 2026

- Base Case U.S. Market Opportunity Estimated at 15,000 Patients, With Potential to Expand to Approximately 30,000 Patients -

- On Track for Completion of 4WARD Phase 3 Target Enrollment in Q4 2026 -

*- Planned 4STEPS Phase 2b Trial Evaluating G-CSF Dose Reduction with Mavorixafor;
Initiation Expected in Q1 2027 -*

- Topline Data from Both 4WARD and 4STEPS Expected in Q1 2028 -

BOSTON, Oct. 29, 2026 (GLOBE NEWSWIRE) -- [X4 Pharmaceuticals, Inc.](#) (Nasdaq: XFOR), a company focused on improving the lives of people with rare hematology diseases, today hosted a virtual Investor Day highlighting the U.S. market opportunity for mavorixafor, an investigational, once-daily oral CXCR4 antagonist, in primary chronic neutropenia (PCN). The event featured a clinical program update, new market research, and a fireside chat with key opinion leaders, Shreyans Gandhi, M.D., of King's College Hospital NHS Foundation Trust, London, and Julia Warren, M.D., Ph.D., of Children's Hospital of Philadelphia.

"PCN is a serious, chronic disease with no new therapy approved in more than 30 years, and today we outlined a clear path to potentially bring the first once-daily oral therapy to people living with this disease," said Adam Craig, M.D., Ph.D., Executive Chair of X4 Pharmaceuticals. "Our 4WARD Phase 3 trial is designed to clearly measure the clinical benefit of mavorixafor, and we remain on track to fully enroll the study by year end. Our planned 4STEPS Phase 2b trial is designed to complement 4WARD by evaluating whether granulocyte colony-stimulating factor (G-CSF) dosage can be reduced or discontinued after initiating mavorixafor. Our most recent commercial research supports a U.S. market opportunity of approximately 15,000 patients, with the potential to grow to approximately 30,000. With data from both trials expected in Q1 2028, we look forward to a milestone-rich next 18 months."

Anticipated milestones:

- Complete target enrollment in 4WARD Phase 3 trial: Q4 2026
- First participant enrolled in 4STEPS Phase 2b trial: Q1 2027
- Topline data from both 4WARD and 4STEPS trials: Q1 2028
- If 4WARD results are positive, potential U.S. Food and Drug Administration (FDA) approval of mavorixafor in PCN in late 2028 or early 2029

Market Opportunity Highlights

- PCN is a heterogeneous disease that is often underdiagnosed and misdiagnosed, which X4 believes has resulted in a large, underserved patient population.
- Claims data identified approximately 15,000 symptomatic U.S. patients aged 12 and older with an absolute neutrophil count (ANC) below 1,000 cells/ μ L and two or more infections in 2025. This base-case population is consistent with patients enrolled in the 4WARD Phase 3 trial.
- Claims data has also identified a potential expansion opportunity, subject to FDA labeling, of 30,000 U.S. patients with an ANC below 1,000 cells/ μ L and one or more infections in 2025.
- Current treatment options are limited to G-CSF injections and antibiotics, and X4 believes a significant unmet medical need remains despite these therapies.
- There is currently no approved oral therapy for PCN, and patients may rely on off-label or burdensome treatments.

Clinical Program Update

- Completion of target enrollment of 126 participants in 4WARD is expected in the fourth quarter of 2026. Blinded, pooled baseline characteristic data as of September 2026 show a median ANC of 483 cells/ μ L, with 68% of participants having three or more infections at baseline.
- X4 introduced 4STEPS, a planned Phase 2b trial 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.

- Topline data from both 4WARD and 4STEPS trials are expected in the first quarter of 2028.
- If positive, the 4WARD results could support a regulatory submission for mavorixafor in PCN, with potential FDA approval in late 2028 or early 2029.

Webcast Replay

A replay of the Investor Day webcast and the accompanying slide presentation are available in the [Events & Presentations](#) section of the X4 Investor Relations website [here](#).

About Mavorixafor

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. Mavorixafor is approved in the U.S. and the European Union as XOLREMDI® for the treatment of patients 12 years and older with WHIM syndrome. Mavorixafor is also being evaluated for the treatment of primary chronic neutropenia in a Phase 3 clinical trial. The FDA has granted Fast Track designation to mavorixafor for the treatment of chronic neutropenia.

About Primary Chronic Neutropenia

Primary chronic neutropenia (PCN) is a 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 PCN 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. PCN is a heterogeneous disease that includes idiopathic, congenital, cyclic, and autoimmune forms, and is often underdiagnosed and misdiagnosed.

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 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 design of, and timing of completion of target enrollment in, the 4WARD trial; the design, initiation, enrollment and timing of the planned 4STEPS trial; the timing of topline data from 4WARD and 4STEPS; the potential regulatory approval of mavorixafor in chronic neutropenia and the timing thereof; the size of the potential market opportunity and addressable patient population for mavorixafor in PCN, including potential expansion opportunities; the potential therapeutic benefits and commercial potential of mavorixafor, including the potential for mavorixafor to be the first once-daily oral therapy for PCN and to permit reduction or discontinuation of G-CSF; X4’s anticipated milestones; and other 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 risks related to the initiation, enrollment, conduct, timing and results of clinical trials; feedback from regulatory authorities; the limitations of market research and claims data analyses; X4’s ability to obtain sufficient capital; 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 from time to time, including its Quarterly Reports on Form 10-Q. 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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