X4 Pharmaceuticals Announces Upcoming Presentation of Phase 2 Chronic Neutropenia Trial Data at the 30th Annual EHA Congress

May 14, 2025

Meaningful and durable increases in functional neutrophils observed over 6-month trial with oral once-daily mavorixafor

Majority of physicians and chronic neutropenia participants chose to significantly reduce G-CSF dosing when using mavorixafor in combination therapy

BOSTON, May 14, 2025 (GLOBE NEWSWIRE) -- **X4 Pharmaceuticals** (Nasdaq: XFOR), a company driven to improve the lives of people with rare diseases of the immune system, today announced the acceptance of two abstracts for poster presentation at the 30th Annual Congress of the European Hematology Association (EHA) taking place in Milan, Italy from June 12-15, 2025. The abstracts published online today summarize the clinical results from the company's completed Phase 2 trial of mavorixafor in the treatment of certain primary chronic neutropenic (CN) disorders.

"We are excited to present the positive data from our completed Phase 2 trial of chronic treatment of CN participants with mavorixafor to the global hematology community for the first time," said Christophe Arbet-Engels, MD, PhD, Chief Medical Officer of X4 Pharmaceuticals. "With enrollment progressing in our ongoing pivotal Phase 3 trial in CN, we continue to look at these Phase 2 results as supportive of the design of the Phase 3 trial and a signal of its potential for success. Mavorixafor is the first innovation to be studied for the treatment of CN in more than 30 years and would be the first oral treatment available for this serious condition if approved."

Summary of EHA Posters to be Presented:
The Phase 2 study of oral, once-daily mavorixafor was a six-month, open-label clinical trial that enrolled a total of 23 participants diagnosed with idiopathic, congenital, or cyclic chronic neutropenia. The two poster presentations at EHA will focus on clinical results based on the following goals of the study:

- Assessing safety and the ability of oral, once-daily mavorixafor to sustainably raise absolute neutrophil count (ANC) as a monotherapy and in combination with injectable granulocyte colony-stimulating factor (G-CSF); and
- Assessing whether treatment of participants with mavorixafor could enable the reduction of G-CSF dosage while maintaining clinically targeted ANC levels and physicians' willingness to do so.

Evaluating the Safety and Efficacy of Mavorixafor, an Oral CXCR4 Antagonist, in Patients with Chronic Neutropenic Disorders: Results from the Phase 2 Study
Lead Author: Julia T. Warren, MD, PhD, Department of Pediatrics, Division of Hematology, Perelman School of Medicine, University of Pennsylvania, and Division of Pediatric Hematology, Children's Hospital of Philadelphia
Poster #: PS1654
Poster Session: Bone marrow failure syndromes incl. PNH - Clinical
Date Available: Thursday, June 12

Mavorixafor, an Oral CXCR4 antagonist, Allows for Granulocyte-Colony Stimulating Factor Dose De-escalation in Patients with Chronic Idiopathic Neutropenia and Congenital Neutropenia
Lead Author: Julia T. Warren, MD, PhD, Department of Pediatrics, Division of Hematology, Perelman School of Medicine, University of Pennsylvania, and Division of Pediatric Hematology, Children's Hospital of Philadelphia
Poster #: PS1669
Poster Session: Bone marrow failure syndromes incl. PNH - Clinical
Date Available: Thursday, June 12

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, increased risk of infections, and reduced quality of life. 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 Phase 1b/Phase 2 Chronic Neutropenia Trial
The Phase 1b/Phase 2 clinical trial (NCT04154488) was a proof-of-concept, open-label, multicenter study designed to assess oral mavorixafor, with or without injectable G-CSF, in participants with chronic neutropenic disorders, including idiopathic, cyclic, and congenital neutropenia. In the Phase 1b portion of the study (n=25), participants received one dose of oral mavorixafor and were assessed for magnitude of absolute neutrophil count (ANC) response and tolerability. The Phase 2 portion of the trial (n=23) assessed the safety, tolerability, and the impact on participants' neutropenia of oral, once-daily mavorixafor with and without concurrent injectable G-CSF therapy over a six-month period in the same patient population.

About the 4WARD Global, Pivotal Phase 3 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 people 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 150 participants 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 endpoint of the trial is based on two outcome measures: 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 diseases of the immune system and significant unmet needs. Leveraging expertise in CXCR4 and immune system biology, 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 people 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 initiation, timing, progress, and results of our current and future preclinical studies and clinical trials.

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: trials, studies and research programs may be delayed and may not have satisfactory outcomes, additionally earlier trials and studies may not be predictive of later trials and studies, including assessing the ability of mavorixafor monotherapy to durably increase absolute neutrophil count in patients with chronic neutropenic; the design and rate of enrollment for current clinical trials may not enable successful completion of the trial(s); X4 may be unable to obtain and maintain regulatory approvals; adverse safety effects may arise from the testing or use of X4’s product and product candidates; 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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