X4 Pharmaceuticals Presents Positive Phase 2 Chronic Neutropenia Trial Data in Poster Presentations at the 30th Annual Congress of the European Hematology Association (EHA)

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Poster #1654 highlights clinically meaningful and durable increases in circulating neutrophils observed with mavorixafor treatment +/- G-CSF over 6-month study

Poster #1669 highlights findings that investigators were willing and able to reduce injectable G-CSF while maintaining ANC levels in the normal range

Data continue to strengthen confidence in potential success of ongoing global, pivotal Phase 3 trial evaluating mavorixafor in primary chronic neutropenia

BOSTON, June 16, 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 presentation of positive data from its completed Phase 2 trial of mavorixafor in the treatment of certain chronic neutropenic (CN) conditions at the 30th Annual Congress of the European Hematology Association (EHA) in Milan, Italy.

"We are very proud to have sponsored this trial – the first of its kind – evaluating an oral agent for the chronic treatment of certain primary chronic neutropenic disorders," said Christophe Arbet-Engels, M.D., Ph.D., Chief Medical Officer of X4 Pharmaceuticals. "In addition to helping us design our ongoing Phase 3 pivotal clinical trial of mavorixafor in CN, the trial also provided a first glimpse into the potential real-world use of mavorixafor in a market currently served by only one approved therapy, with both clinical investigators and participants willing and then able to significantly reduce or discontinue the dose of injectable granulocyte colony-stimulating factor, or G-CSF. The results from this Phase 2 study continue to strengthen our confidence in a successful conclusion to our Phase 3 trial, which we expect will fully enroll in the third or fourth quarter of this year."

Julia A. Warren, M.D., Ph.D., study investigator and hematologist within both the Department of Pediatrics, Division of Hematology, Perelman School of Medicine, University of Pennsylvania, and the Division of Pediatric Hematology, Children's Hospital of Philadelphia, commented on the results: "I was pleased to participate in this clinical trial and to present the results at this year's EHA meeting. I am grateful to the participants with neutropenia who are currently enrolling in the Phase 3 clinical trial, as this rigorous study will help us understand which neutropenia patients will ultimately benefit from this drug should it gain regulatory approval."

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, and had two subsets: mavorixafor monotherapy (n = 10 at baseline) and mavorixafor in combination with injectable G-CSF (n=13 at baseline). The two poster presentations at EHA today focused 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 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.

Key Findings from the Poster Presentations:

- Results from participants receiving oral, once-daily mavorixafor monotherapy showed that mavorixafor durably increased mean ANC from baseline over the 6-month trial.
    - Further analysis showed that those with severe CN achieved nearly 3-fold increases in mean ANC levels out to six months, reaching levels typically targeted by physicians for patients with severe chronic neutropenia.
- The majority of participants (89%) and investigators were willing and elected to substantially reduce or discontinue injectable G-CSF use with oral mavorixafor treatment. All participants with congenital neutropenia concurrently treated with G-CSF, including one with the *ELANE* variant, were able to decrease G-CSF while maintaining mean ANC at normal levels.
    - This study provides the first evidence that a significant subset of patients with primary CN may be able to successfully transition off injectable G-CSF to mavorixafor, potentially providing the first oral option to treat chronic neutropenia.
- In addition, a sub-study comparing the mean percentage of functional neutrophils in samples from healthy donors (n=5) to participants from the Phase 2 CN study (n=9) showed that the mean percentage of functional circulating neutrophils in CN participants in this sub-study was comparable to that of healthy donors after six months of mavorixafor dosing.

- Mavorixafor was generally well tolerated as monotherapy and in combination with injectable G-CSF during the trial, with no drug-related serious adverse events reported; the overall safety profile observed in the study was consistent with previous clinical studies of mavorixafor.

X4 is currently conducting a global, pivotal Phase 3 trial in chronic neutropenia to assess the ability of mavorixafor to safely and durably increase ANC levels while also reducing participants' annualized infections rates over a 52-week period.

About Chronic Neutropenia and Mavorixafor
Chronic neutropenia is a rare blood condition lasting more than three months and characterized by persistently or intermittently 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, enrollment progress, and results of our current and future preclinical studies and clinical trials, including our ongoing pivotal Phase 3 clinical trial; the timing and period during which the results of the trials will become available and reported, as well as our research and development programs; and expectations regarding the commercial potential of mavorixafor and ongoing engagement and feedback from regulatory authorities.

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 expected availability, content, and timing of clinical data from X4's ongoing clinical trials of mavorixafor may be delayed or unavailable, including the ongoing Phase 3 clinical trial; 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; the need to align with collaborators may hamper or delay development and commercialization efforts or increase costs; and business may be adversely affected and clinical trials may be delayed if any of the company's key collaborators fails to perform its obligations or terminates the collaboration; 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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