X4 Pharmaceuticals Granted Fast Track Designation for Mavorixafor for the Treatment of Chronic Neutropenia by U.S. FDA

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BOSTON, June 10, 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 that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to mavorixafor, an oral CXCR4 antagonist, for the treatment of chronic neutropenia (CN). The company is currently conducting a global, pivotal Phase 3 clinical trial (4WARD) evaluating mavorixafor in certain primary CN conditions. Mavorixafor was previously granted Fast Track designation for the treatment of WHIM syndrome, a rare immunodeficiency indication for which it received FDA approval in April 2024.

"We are thrilled that the FDA has recognized the unmet needs in the chronic neutropenia community and granted us Fast Track designation for mavorixafor for the treatment of CN," said Christophe Arbet-Engels, M.D., Ph.D., Chief Medical Officer of X4 Pharmaceuticals. "We look forward to working with the FDA and leveraging the benefits of the designation, which include more frequent communication with the agency and the potential for accelerated approval and/or priority review if certain criteria are met and maintained. With enrollment ongoing in our Phase 3 4WARD trial of mavorixafor in CN, we continue to expect full enrollment in the third or fourth quarter of this year and potential top-line data in late 2026."

At present, the only FDA-approved treatment for CN is injectable human recombinant granulocyte-colony stimulating factor (G-CSF), approved in the 1990s for severe chronic neutropenia. G-CSF is associated with side effects that include bone pain, splenomegaly, thrombocytopenia, glomerulonephritis, vasculitis, and osteoporosis, and long-term treatment, especially at high doses, is correlated with an increased risk of leukemia in patients with congenital neutropenia. Due to these often dose-limiting toxicities and risks associated with long-term use, patients do not always receive the optimal dosage regimen on G-CSF. The company believes that oral mavorixafor has the potential to benefit those with certain primary CN conditions by offering a better balance of disease control and burden of treatment.

The FDA's Fast Track process is designed to facilitate and streamline the development and expedite the review of drugs that have demonstrated the potential to address unmet medical needs in the treatment of a serious or life-threatening disease of condition.

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 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 injectable granulocyte colony-stimulating factor, 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 and the potential topline data; 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 feedback from regulatory authorities, including Fast Track designation and its potential benefits, and the potential for accelerated approval and/or priority review by the FDA.

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 and potential topline data; 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 CN; 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, including meeting the Fast Track designation criteria required to be granted the potential for accelerated approval and/or priority review by the FDA; adverse safety effects may arise from the testing or use of X4's product and product candidates; X4 may not be able to establish the potential improved balanced disease control and burden of treatment with oral mavorixafor for patients with certain primary CN conditions; 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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