X4 Pharmaceuticals Announces Fast Track Designation Granted by the FDA to Mavorixafor for the Treatment of WHIM Syndrome

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BOSTON, Oct. 08, 2020 (GLOBE NEWSWIRE) -- **X4 Pharmaceuticals, Inc.** (Nasdaq: XFOR), a leader in the discovery and development of novel therapies targeting diseases resulting from dysfunction of the CXCR4 pathway, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track Designation to its lead asset, mavorixafor, for the treatment of adult patients with WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) syndrome, a rare, inherited, primary immunodeficiency disease caused by genetic mutations in the CXCR4 receptor gene.

Mavorixafor is a potential first-in-class, once-daily, oral, small molecule antagonist of chemokine receptor CXCR4, currently being investigated in a global pivotal Phase 3 clinical trial, 4WHIM, for the treatment of WHIM syndrome.

“The Fast Track Designation of mavorixafor for the treatment of WHIM syndrome further recognizes WHIM as a serious condition with a clear unmet need for an effective and potentially disease-modifying therapy,” said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. “We recently showed the potential therapeutic benefit of mavorixafor in a Phase 2 clinical trial in WHIM, with significant increases in white blood cell counts and reductions in infection rates and wart burden. We look forward to continuing to demonstrate mavorixafor’s clinical utility in WHIM syndrome in our ongoing Phase 3 clinical trial and working with the FDA to potentially bring this promising therapy to patients as soon as possible.”

The Fast Track Designation aims to facilitate the expedited development and review of new drugs that are intended to treat serious or life-threatening conditions and demonstrate the potential to address unmet medical needs. Through the Fast Track program, X4 will be eligible for more frequent meetings with the FDA to discuss the drug’s development plan, protocols and clinical data that would support mavorixafor’s potential approval for WHIM.

In addition to Fast Track Designation, mavorixafor was previously granted Breakthrough Therapy Designation by the FDA, as well as Orphan Drug status by the FDA and the European Commission for the treatment of WHIM syndrome.

About Mavorixafor in WHIM Syndrome
WHIM syndrome is a rare, inherited, primary immunodeficiency disease caused by gain-of-function mutations in the chemokine receptor CXCR4, resulting in a reduced mobilization and trafficking of white blood cells from the bone marrow. The company estimates there to be more than 3,500 diagnosed and undiagnosed WHIM patients in the U.S. As a first-in-class, small-molecule antagonist of chemokine receptor CXCR4, mavorixafor is designed to address the underlying cause of WHIM directly. The candidate is currently being developed as a once-daily oral therapy in the Phase 3 4WHIM trial, a 52-week, randomized, double-blind, placebo-controlled, multicenter study designed to evaluate the safety and efficacy of mavorixafor in genetically confirmed WHIM patients. The trial is anticipated to enroll up to 28 subjects in

approximately 20 countries, followed by an open-label extension trial. Phase 3 results are expected in 2022.

About X4 Pharmaceuticals

X4 Pharmaceuticals is a late-stage clinical biopharmaceutical company and a leader in the discovery and development of novel therapies for the treatment of diseases resulting from dysfunction of the CXCR4 pathway, with a focus on rare diseases and those with limited treatment options. The company's lead candidate, mavorixafor, is a first-in-class, small molecule antagonist of chemokine receptor CXCR4 being developed as a once-daily oral therapy. X4 believes that inhibition of the CXCR4 receptor creates the potential for mavorixafor to provide therapeutic benefit across a wide variety of diseases, including primary immunodeficiencies and certain types of cancer. The efficacy and safety of mavorixafor, dosed once daily, is currently being evaluated in a global Phase 3 clinical trial in patients with WHIM syndrome, and in two Phase 1b clinical trials – in combination with ibrutinib in patients with Waldenstrom macroglobulinemia, and as monotherapy in patients with severe congenital neutropenia (SCN). X4 is continuing to leverage its insights into CXCR4 biology at its corporate headquarters in Boston, Massachusetts and at its research facility in Vienna, Austria, and is developing additional product candidates. For more information, please visit **www.x4pharma.com**.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of 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, statements regarding the clinical development of mavorixafor and X4's other product candidates or programs. Any forward-looking statements in this press release are based on management's current expectations and beliefs. Actual events or results may differ materially from those expressed or implied by any forward-looking statements contained herein, including, without limitation, the risks and uncertainties described in the section entitled "Risk Factors" in X4's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on August 4, 2020, and in other filings X4 makes with the SEC 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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