X4 Pharmaceuticals Receives Rare Pediatric Disease Designation from FDA for Mavorixafor for the Treatment of WHIM Syndrome

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BOSTON, Dec. 10, 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 it has received Rare Pediatric Disease (RPD) Designation from the U.S. Food and Drug Administration (FDA) for its lead asset, mavorixafor, for the treatment of WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) syndrome, a rare, inherited, primary immunodeficiency disease caused by genetic mutations in the CXCR4 receptor gene. Mavorixafor is currently being investigated in a global pivotal Phase 3 clinical trial, 4WHIM, for the treatment of WHIM syndrome in patients who are 12 years of age and older.

“WHIM is a congenital disease that affects individuals of all ages. Children, in particular, have been shown to experience serious or life-threatening bacterial infections that can require hospitalizations. There are currently no treatments available that address the underlying genetic cause of WHIM,” said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. “The RPD designation reinforces the clear unmet need for a disease modifying therapy in both pediatric and adult patient populations.”

RPD designations are granted by the FDA for serious or life-threatening diseases in which the serious or life-threatening manifestations primarily affect individuals between birth and 18 years of age. Under the RPD program, a sponsor who receives an approval for a drug for a “rare pediatric disease” and a Fast Track designation may qualify for a voucher that can be redeemed to receive a priority review by the FDA for any subsequent marketing application for a different product. Such a voucher is transferrable and may be sold.

Mavorixafor had previously been granted Fast Track Designation and Breakthrough Therapy Designation by the FDA, as well as Orphan Drug status by the FDA and the European Commission (EC), for the treatment of WHIM syndrome in adults.

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, and the potential benefits resulting from a Rare Pediatric Disease designation. 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 November 5, 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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