X4 Pharmaceuticals Completes Enrollment in Phase 3 Mavorixafor Trial in Patients with WHIM Syndrome

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Final enrollment of 31 adult and pediatric patients exceeds initial target of 18-28 patients

Top-line data expected in 4Q 2022; U.S. NDA filing anticipated in 1Q 2023

BOSTON, Oct. 04, 2021 (GLOBE NEWSWIRE) -- **X4 Pharmaceuticals, Inc.** (Nasdaq: XFOR), a leader in the discovery and development of novel CXCR4-targeted small molecule therapeutics to benefit patients with diseases of the immune system, announced today that it has completed enrollment in the ongoing pivotal Phase 3 clinical trial of its lead candidate, mavorixafor, in the treatment of patients with genetically confirmed WHIM (warts, hypogammaglobulinemia, infections, and myelokathexis) syndrome, a primary immunodeficiency caused by gain-of-function mutations in the CXCR4 gene. Thirty-one adult and pediatric patients have enrolled in the **4WHIM** trial, which compares a once-daily, oral dose of mavorixafor to placebo across primary and secondary endpoints that include clinically relevant counts of neutrophils and lymphocytes, the frequency and severity of both infections and warts, as well as certain quality of life measurements. The trial was originally designed to enroll 18-28 patients.

"This major milestone achievement for X4 is a hopeful step forward for the thousands of WHIM patients with no disease-modifying treatment options," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4. "We would like to especially recognize and thank the patient participants in the 4WHIM trial and their families and caregivers, our study investigators and their clinical teams, as well as our vendors, contractors, and employees who made this first-of-its-kind accomplishment possible."

Dr. Ragan continued: "As we now look ahead to announcing top-line data from the 4WHIM trial, expected in the fourth quarter of 2022, and to a possible regulatory filing in the first quarter of 2023, we are turning our focus towards pre-commercialization efforts, including further identifying WHIM patients and the physicians most likely to care for them. In addition, we look forward to the availability of new long-term data from our ongoing Phase 2 open-label extension trial in WHIM as well as new research and clinical data later this year that we anticipate will support broader market opportunities for mavorixafor across multiple indications."

About Mavorixafor and WHIM Syndrome
WHIM (warts, hypogammaglobulinemia, infections, and myelokathexis) syndrome is a rare, autosomal-dominant, inherited, primary immunodeficiency disease caused by gain-of-function mutations in the chemokine receptor CXCR4 that result in reduced mobilization and trafficking of white blood cells from the bone marrow. Patients with WHIM syndrome typically experience frequent, recurrent infections with a high risk of lung disease, refractory warts from underlying human papillomavirus (HPV) infection, limited antibody production due to low levels of immunoglobulin, and an increased risk of developing certain types of cancer. Mavorixafor is an investigational, first-in-class, small-molecule antagonist of CXCR4 being developed as a once-daily oral therapy to correct the dysfunction resulting from the underlying genetic causes of WHIM. In more than 200 clinical subjects to date, mavorixafor has shown good tolerability and the ability to mobilize neutrophils, lymphocytes, and monocytes out of the bone marrow. For the WHIM indication, mavorixafor has been granted Breakthrough Therapy Designation, Fast Track Designation, and Rare Pediatric Designation in the U.S., and Orphan Drug Status in both the U.S. and European Union.

About the 4WHIM Clinical Trial
The 4WHIM Phase 3 clinical trial (NCT03995108) is a global, randomized, double-blind, placebo-controlled, multicenter study designed to evaluate the efficacy and safety of mavorixafor in genetically confirmed WHIM patients. Originally designed to enroll 18-28 patients, the trial has enrolled 31 patients aged 12 and older who are receiving either 400 mg mavorixafor or placebo orally once daily for 52 weeks; all patients then become eligible to receive treatment with mavorixafor in an open-label trial extension. The primary endpoint of the 4WHIM trial is a clinically relevant reduction of severe neutropenia as measured by the increase in time above threshold (500 cells per microliter) for the absolute neutrophil count (or "TAT-ANC") in peripheral blood. Secondary endpoints include change from baseline in total absolute lymphocyte count, absolute monocyte count, and white blood cells; change from baseline in cutaneous warts and infection rates at 52 weeks; and a number of quality-of-life measurements and other exploratory endpoints.

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 of the immune system via antagonism 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 number of clinical trials, including 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 Waldenström's macroglobulinemia, and as monotherapy in patients with Severe Congenital Neutropenia (SCN) and other chronic neutropenia disorders. 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 discovering and 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 and therapeutic potential of mavorixafor; X4’s potential growth and evolution; the advancement of X4’s pipeline; the potential WHIM patient population; and the potential commercialization of mavorixafor and any other of X4’s product candidates, if approved. 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, uncertainties inherent in the initiation and completion of preclinical studies and clinical trials and clinical development; the risk that trials and studies may be delayed, including, but not limited to, as a result of the effects of the ongoing COVID-19 pandemic or delayed patient enrollment, and may not have satisfactory outcomes; the risk that the outcomes of preclinical studies or earlier clinical trials will not be predictive of later clinical trial results; the potential adverse effects arising from the testing or use of mavorixafor or other product candidates; risks related to X4’s ability to raise additional capital and other risks and uncertainties, including those 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 3, 2021, 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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