X4 Pharmaceuticals Announces Late-Breaking Abstract of WHIM Phase 3 Clinical Data Accepted for Oral Presentation at the 2023 Annual Meeting of the Clinical Immunology Society (CIS)

April 12, 2023

X4 to host virtual investor event on Tuesday, May 16

CIS oral presentation to take place on Sunday, May 21

BOSTON, April 12, 2023 (GLOBE NEWSWIRE) -- **X4 Pharmaceuticals, Inc.** (Nasdaq: XFOR), a leader in the discovery and development of novel small molecule therapeutics to benefit patients with diseases of the immune system, today announced that its submitted late-breaker abstract entitled "*Results of a Phase 3 Trial of an Oral CXCR4 Antagonist, Mavorixafor, for Treatment of Patients With WHIM Syndrome*" has been accepted for oral presentation at the Annual Meeting of the Clinical Immunology Society (CIS), which is taking place May 18-21, 2023, in St. Louis, MO. New data related to clinical secondary endpoints, among other assessments, are to be presented.

In addition, X4 announced that the company will host a virtual event to present and discuss new data from the 4WHIM Phase 3 clinical trial at 4:00 pm ET on Tuesday, May 16, following expected publication of conference abstracts. In November 2022, X4 announced that the 4WHIM trial had met its primary endpoint and a key secondary endpoint, and that mavorixafor was generally well tolerated in the trial, with no treatment-related serious adverse events reported and no discontinuations for safety events. New data to be presented during the event will focus on additional secondary endpoints, including the impact of oral, once-daily mavorixafor on the rate, severity, and duration of infections, among other outcomes metrics, as measured during the 52-week trial period.

Additionally, the company expects to provide an update on its U.S. regulatory activities in support of its potential New Drug Application-submission for mavorixafor for the treatment of WHIM syndrome, which is expected early in the second half of 2023.

The X4 live-event webcast will be accessible on the investor relations section of the X4 website at www.x4pharma.com. Following the completion of the event, a replay will be available on the website.

At the CIS Conference, Dr. Raffaele Badolato, Professor of Pediatrics at the University of Brescia (Italy) and an investigator in the 4WHIM clinical trial, will present at 11:30 am CT on Sunday, May 21. Although the session will only be accessible live to conference attendees, X4 will post the slides on its website concurrent with the presentation.

About Mavorixafor and WHIM Syndrome
WHIM (warts, hypogammaglobulinemia, infections, and myelokathexis) syndrome is a rare, inherited, combined immunodeficiency disease caused by reduced mobilization and trafficking of white blood cells from the bone marrow due to over-signaling of the CXCR4/CXCL12 pathway. People with WHIM syndrome characteristically have very low blood levels of neutrophils (neutropenia) and lymphocytes (lymphopenia), and as a result, 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 small-molecule antagonist of CXCR4 being developed as a once-daily oral therapy to correct the dysfunction resulting from the underlying causes of WHIM. 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 Phase 3 Clinical Trial
The 4WHIM Phase 3 clinical trial was a global, randomized, double-blind, placebo-controlled, multicenter study designed to evaluate the efficacy and safety of oral, once-daily mavorixafor in people with genetically confirmed WHIM syndrome. Originally designed to enroll 18-28 patients, the trial enrolled 31 patients aged 12 and older who received either 400 mg mavorixafor (n=14) or placebo (n=17) orally once daily for 52 weeks.

About X4 Pharmaceuticals
X4 Pharmaceuticals is a late-stage clinical biopharmaceutical company focused on the discovery and development of novel therapies for people with diseases of the immune system. Our lead clinical candidate is mavorixafor, a small molecule antagonist of chemokine receptor CXCR4 that is being developed as an oral, once-daily therapy. Due to its ability to increase the mobilization of mature, functional white blood cells from the bone marrow into the bloodstream, we believe that mavorixafor has the potential to provide therapeutic benefit across a variety of chronic neutropenic disorders, including WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) syndrome, a rare, primary immunodeficiency. Following announcement of positive top-line data from our global, pivotal, 4WHIM Phase 3 clinical trial, we are currently preparing a U.S. regulatory submission seeking approval of oral, once-daily mavorixafor in the treatment of people aged 12 years and older with WHIM syndrome. We are also currently evaluating mavorixafor in a Phase 2 clinical trial in people with certain chronic neutropenic disorders following positive results from a Phase 1b clinical trial of mavorixafor in people with congenital, idiopathic, or cyclic neutropenia. We continue to leverage our insights into CXCR4 and immune system biology at our corporate headquarters in Boston, Massachusetts and at our research center of excellence in Vienna, Austria. For more information, please visit our website at www.x4pharma.com.

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, statements regarding the clinical progress of X4’s pipeline development programs, including the anticipated New Drug Application submission for mavorixafor for the treatment of WHIM syndrome and the timing thereof. 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, on account of uncertainties inherent in the initiation and completion of clinical trials and clinical development; the risk that trials and studies 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 risk that initial or interim results from a clinical trial may not be predictive of the final results of the trial or the results of future trials; the potential adverse effects arising from the testing or use of mavorixafor or other product candidates; the risk that the Food and Drug Administration (FDA) may not support and accept a regulatory submission for mavorixafor, and that X4’s interactions with the FDA may not have satisfactory outcomes; the risks related to X4’s ability to raise additional capital; the impacts of macroeconomic conditions, including the COVID-19 pandemic, the conflict in Ukraine, rising inflation, and uncertain credit and financial markets on X4’s business, clinical trials and financial position; and other risks and uncertainties, including those described in the section entitled “Risk Factors” in X4’s Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on March 21, 2023, 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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