X4 Pharmaceuticals to Present Research Data at the 2022 European Hematology Association (EHA) Congress Supporting Mechanism and Market Potential for Mavorixafor

May 16, 2022

BOSTON, May 16, 2022 (GLOBE NEWSWIRE) -- **X4 Pharmaceuticals, Inc.** (Nasdaq: XFOR), a leader in the discovery and development of novel CXCR4-targeted small molecule therapeutics to benefit people with rare immune system disorders, today announced that it will present research data supporting the mechanism and market potential of its lead drug candidate, mavorixafor, as part of three posters at the upcoming European Hematology Association (EHA) congress to be held in Vienna, Austria, June 9-17, 2022.

Mavorixafor is an oral, small molecule, CXCR4 antagonist currently being evaluated across three clinical studies: a fully enrolled Phase 3 trial in adults and adolescents with WHIM (Warts, Hypogammaglobulinemia, Infections, Myelokathexis) syndrome; a Phase 1b clinical trial in adults with chronic neutropenia; and a fully enrolled Phase 1b clinical trial in adults with Waldenström's macroglobulinemia (WM), a rare B-cell lymphoma.

"We are excited to present new data supporting our ongoing efforts to further elucidate correlations between clinical presentation and new genetic variants associated with WHIM syndrome," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "These data continue to enhance our ability to identify undiagnosed patients, including those who may potentially benefit from treatment with mavorixafor. In addition, we believe that data on the mechanism of action of mavorixafor in the WM tumor environment continues to demonstrate its potential in this difficult-to-treat patient population. We look forward to presenting additional data throughout 2022 from our ongoing research and clinical studies and to advancing our efforts to improve the lives of people with diseases that could benefit from CXCR4 antagonism."

Key highlights:

- WHIM genetics: Identification of 17 new CXCR4 genetic variants that caused *in vitro* functional defects resembling those exhibited by known WHIM variants, with potential to enrich current estimates of WHIM syndrome prevalence.
- Development of first cell-based model recapitulating heterozygous $CXCR4^{WT/R334X}$ mutations found in patients with WHIM syndrome, which enriches the toolbox of models available for studying WHIM's biology and treatment options.
- WM: Study provides preliminary evidence for the potential mechanism of mavorixafor in disrupting the interaction of WM cells with the tumor microenvironment, enhancing the efficacy of B-cell targeted therapies in the treatment of WM and potentially other lymphomas.

Poster Details:

Poster Title: Screening of Naturally Occurring CXCR4 Variants for Identification of Novel Pathogenic Mutations for WHIM Syndrome

Poster #: P793
Session Date/Time: June 10 at 16:30 – 17:45 CEST

Poster Title: Crispr/Cas9-Based Model of Heterozygous CXCR4$^{WT/R334x}$ Mutation to Study Cellular Phenotypes in WHIM Syndrome
Poster #: P792
Session Date/Time: June 10 at 16:30 – 17:45 CEST

Poster Title: Mavorixafor Disrupts the Cross Talk Between Waldenström's Macroglobulinemia Cells and the Bone Marrow Microenvironment Resulting in Enhanced Sensitivity to B-Cell Targeted Therapies
Poster #: P1253
Session Date/Time: June 10 at 16:30 – 17:45 CEST

A copy of the posters will be available on X4's corporate website at the conclusion of the conference. Additionally, an abstract detailing the 4WHIM Phase 3 trial patient demographics and neutropenia severity has also been accepted for publication only at EHA.

About X4 Pharmaceuticals
X4 Pharmaceuticals is a late-stage clinical biopharmaceutical company leading the discovery and development of novel therapies for people with rare diseases of the immune system. The company's lead candidate is mavorixafor, a first-in-class, small molecule antagonist of chemokine receptor CXCR4 that is being developed as a once-daily oral therapy. Due to mavorixafor's ability to antagonize CXCR4 and improve the healthy maturation and trafficking of white blood cells, X4 believes that mavorixafor has the potential to provide therapeutic benefit across a wide variety of diseases, including primary immunodeficiencies (PIDs) and certain types of cancer. Mavorixafor has already demonstrated clinical potential in a Phase 2 trial in people with WHIM syndrome, a rare PID. Its efficacy and safety continue to be evaluated in a global Phase 3 clinical trial in WHIM (fully enrolled) and in two Phase 1b clinical trials – one, as monotherapy in people with chronic neutropenia, including Severe Congenital Neutropenia (SCN), and another in combination with ibrutinib in people with Waldenström's macroglobulinemia (also fully enrolled). 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, to discover and develop 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 and X4's other product candidates or programs; X4's possible exploration of additional opportunities for mavorixafor; the anticipated achievement of upcoming clinical milestones; the expected availability, content, and timing of clinical trial data; anticipated regulatory filings and commercial plans; clinical trial design, and the company's cash runway. 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 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; 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 May 12, 2022, and in other filings X4 makes with the SEC from time to time.

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