X4 Pharmaceuticals' Presentations at ASH 2022 Further Highlight Opportunity for Mavorixafor in the Treatment of Chronic Neutropenia

December 12, 2022

Oral presentation further details positive data from mavorixafor Phase 1b clinical trial in people with chronic idiopathic, cyclic, or congenital neutropenia

Medical claims research defines an estimated 50,000 U.S. chronic neutropenia patient population; patient survey voices the significant unmet medical needs

BOSTON, Dec. 12, 2022 (GLOBE NEWSWIRE) -- **X4 Pharmaceuticals** (Nasdaq: XFOR), a leader in the discovery and development of novel small-molecule therapeutics to benefit people with diseases of the immune system, today provided a summary of its chronic neutropenia-related presentations at this year's annual meeting of the American Society of Hematology (ASH), taking place December 10-13, 2022 in New Orleans.

"Our clinical trial results and market research data continue to increase our confidence that mavorixafor could make a meaningful difference in the lives of people with chronic neutropenic disorders," said Paula Ragan, Ph.D., President, and Chief Executive Officer of X4. "In aggregate, our chronic neutropenia-related presentations at this year's ASH meeting not only highlight continued positive clinical data, but also strengthen our belief that there is a sizeable group of diagnosed patients in the U.S. who could benefit from an innovative, oral treatment. We look forward to continuing our advancement of mavorixafor in multiple chronic neutropenic indications and expect additional data in the first half of next year will inform the potential clinical path forward."

In an oral presentation entitled "*Mavorixafor for Patients With Chronic Neutropenic Disorders: Results From a Phase 1b, Open-Label, Multicenter Study,*" Julia T. Warren, M.D., Ph.D., Hematologist at Children's Hospital of Philadelphia and Assistant Professor of Pediatrics at the Perlman School of Medicine at the University of Pennsylvania, presented positive data from a Phase 1b clinical trial evaluating the ability of X4's lead clinical candidate, mavorixafor, to increase absolute neutrophil count (ANC) in people with idiopathic, cyclic, or congenital chronic neutropenia as monotherapy or concurrently with injectable granulocyte colony-stimulating factor (G-CSF).

- Data analyses continue to show that a single dose of oral mavorixafor effected meaningful increases in ANC across all participants, regardless of disease etiology or use of G-CSF.
- Mavorixafor was well tolerated overall in the trial.
- The Phase 1b clinical trial has now been amended and a Phase 2 trial (NCT04154488) is being initiated by X4 to assess the durability, safety, and tolerability of the chronic use of once-daily, oral mavorixafor in a larger chronic neutropenia patient population.

Poster #2407, entitled "*Prevalence of Chronic Neutropenic Disorders in the United States: A Retrospective Analysis of a Large Claims Database,*" estimated that in 2021, between 37,000–48,000 people in the United States were living with a diagnosis of idiopathic, cyclic, or congenital neutropenia.

- The study was a retrospective analysis designed to project the prevalence of chronic neutropenia disorders based on U.S. claims data for people with a diagnosis code for neutropenia during the calendar years 2018, 2019, and 2021. (The year 2020 was excluded from this analysis owing to anticipated reduced claims during the COVID-19 pandemic.)
- People diagnosed with chronic idiopathic, cyclic, or congenital neutropenia were identified using the earliest relevant diagnosis claim based on International Classification of Disease 10th Revision, Clinical Modification (ICD-10-CM) codes in the calendar year of interest.

Poster #3575, entitled "*Patient and Health Care Professional Perspectives on Quality of Life and Unmet Needs of People With Chronic Neutropenia: A Survey-Based Assessment,*" highlighted responses of 100 patients/caregivers and 10 healthcare professionals (HCPs) to a survey on the experiences of people living with chronic neutropenia.

- Survey results demonstrate the considerable impact of chronic neutropenic disorders on the lives of people diagnosed.
- Results also describe the potential burdens related to the existing treatment paradigm, injectable G-CSF, and its limitations to improving physical health and quality of life.
- While patients/caregivers cited fatigue as the most impactful symptom and fatigue reduction as a priority treatment need, HCPs ranked fewer and/or less frequent severe infections requiring hospitalization, how medication is administered, and fewer and/or less frequent long-term side effects as the priority treatment needs.

About the Phase 1b/Phase 2 Clinical Trials

The clinical trial was a proof-of-concept Phase 1b open-label, multicenter study designed to assess the safety and tolerability of oral mavorixafor, with or without G-CSF, in participants with chronic neutropenic disorders, including severe idiopathic, cyclic, and congenital neutropenia. Participants were dosed with a single dose of 400 mg oral mavorixafor to assess the magnitude of treatment response. The Phase 1b clinical trial has now been amended to continue as a Phase 2 clinical trial aiming to evaluate the use of daily oral mavorixafor with or without G-CSF for 6 months in participants with chronic neutropenic disorders.

About Chronic Neutropenia
Chronic neutropenic disorders are rare blood conditions lasting more than three months, persistently or intermittently, and characterized by increased risk of infections and reduced quality of life due to persistent, abnormally low levels of neutrophils circulating in the blood. Chronic neutropenia can be described by a number of etiologies, including idiopathic (of unknown origin), cyclic (typically a 21-day cycle), or congenital (of genetic causation). Neutrophils are retained in the bone marrow by the CXCL12/CXCR4 axis, creating a reserve of cells; downregulation of the CXCR4 receptor by G-CSF or inhibition of the receptor by a CXCR4 antagonist has been shown to mobilize neutrophils from the bone marrow into peripheral blood.

About X4 Pharmaceuticals
X4 Pharmaceuticals is a late-stage clinical biopharmaceutical company leading 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 mavorixafor's ability to antagonize CXCR4 and improve the mobilization of white blood cells, we believe that mavorixafor has the potential to provide therapeutic benefit across a variety of immune system diseases, including a range 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 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 advancing mavorixafor into a Phase 2 clinical trial in people with chronic neutropenic disorders following positive results from a Phase 1b clinical trial of mavorixafor in people with congenital, idiopathic, and 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 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, express or implied statements regarding the therapeutic potential and benefits of mavorixafor for the treatment of patients with chronic neutropenic disorders, including its potential advantages as compared to existing treatment paradigm; the estimated chronic neutropenia patient population in the United States; timing expectations for additional release of data from the Phase 1b/2 clinical trial evaluating mavorixafor in the treatment of patients with chronic neutropenic disorders; and the expectations surrounding clinical development and potential regulatory submissions for mavorixafor, including regulatory approvals and 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 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 FDA may not support and accept a regulatory submission for mavorixafor, and X4's interactions with the FDA may not have satisfactory outcomes; the risks related to X4's ability to raise additional capital; unexpected litigation or other disputes; unfavorable conditions in the global economy; and other risks and uncertainties, including those described in the section entitled "Risk Factors" in X4's Quarterly Report on Form 10-Q for the quarter ended September 30, 2022 filed with the Securities and Exchange Commission (SEC) on November 3, 2022, 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.

Contacts:
Daniel Ferry (investors)
Managing Director, LifeSci Advisors
daniel@lifesciadvisors.com
(617) 430-7576

Cherilyn Cecchini, M.D. (media)
LifeSci Communications
ccecchini@lifescicomms.com

Source: X4 Pharmaceuticals