X4 Pharmaceuticals Announces Presentation of Additional Data from Mavorixafor Phase 2 Trial in Chronic Neutropenia at ASH 2023

December 9, 2023

Initial data continue to support advancement of mavorixafor into pivotal, global Phase 3 trial in certain chronic neutropenic disorders; company on track to initiate in 1H 2024

X4 expects to present additional CN Phase 2 clinical trial data in 1H 2024

BOSTON, Dec. 09, 2023 (GLOBE NEWSWIRE) -- **X4 Pharmaceuticals** (Nasdaq: XFOR), a company driven to improve the lives of people with rare diseases of the immune system, today announced the presentation of additional preliminary data from its ongoing Phase 2 clinical trial evaluating the safety and efficacy of once-daily oral mavorixafor with or without concurrent treatment with injectable granulocyte-colony stimulating factor (G-CSF) in people diagnosed with idiopathic, cyclic, or congenital neutropenia. Julia T. Warren, M.D., Ph.D., a trial investigator and hematologist in the Division of Hematology at the Children's Hospital of Philadelphia, will be presenting Poster #P1160 today at the annual meeting of the Annual Society of Hematology (ASH).

The poster highlights case studies of the first three participants to complete the 6-month Phase 2 trial, all of whom entered the study on G-CSF therapy. Overall:

- Mavorixafor was generally well tolerated when used in combination with G-CSF, with no serious adverse events reported;
- While 4 infections were reported during the first two months of the trial, no infections were reported during the following 4 months of the trial, despite reductions in G-CSF dosing in two of the three participants;
- The data continue to support the potential of mavorixafor to raise absolute neutrophil counts (ANC) and enable reduction in G-CSF dosing in patients with chronic neutropenia;
- The data informed the design of the planned pivotal, global Phase 3 clinical trial of mavorixafor in certain chronic neutropenic disorders, as well as the selection of ANC and infection as primary endpoints; the trial is expected to initiate in the first half of 2024.

The poster also highlighted results from a recent qualitative Chronic Neutropenia Patient Voice Survey that confirmed previous market research showing respondents' strong interest in reducing G-CSF dosing and/or reducing injection frequency with the addition of an oral medication such as mavorixafor.

"Given that there has been little innovation in the classical hematology space and no innovation for patients with chronic neutropenia for the past 30 years, we are very pleased with the data continuing to emerge from our ongoing CN Phase 2 clinical trial," said Christophe Arbet-Engels, M.D., Ph.D., Chief Medical Officer of X4 Pharmaceuticals. "In the first half of 2024, we look forward to presenting more comprehensive data from this Phase 2 trial, which will include both mavorixafor monotherapy treatment data as well as additional results of participants on mavorixafor/G-CSF combination therapy."

About X4 Pharmaceuticals
X4 Pharmaceuticals is a late-stage clinical biopharmaceutical company driven to improve the lives of people with rare 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 across a variety of immunodeficiencies, including WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) syndrome and certain chronic neutropenic disorders. Following successful completion of a global, pivotal, Phase 3 clinical trial, we are seeking U.S. regulatory approval of oral, once-daily mavorixafor for the treatment of people aged 12 years and older with WHIM syndrome. We are also currently planning a Phase 3 clinical program evaluating mavorixafor in certain chronic neutropenic disorders. 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 development and therapeutic potential of mavorixafor for the treatment of chronic neutropenic disorders; expectations regarding timing for reporting data from ongoing clinical studies or the initiation of future clinical trials, including the timing of reporting additional data from X4's ongoing Phase 2 trial of mavorixafor in certain chronic neutropenic disorders and the timing of commencing a Phase 3 trial. 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 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 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 November 9, 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.

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

Ché Knight (media)
LifeSci Communications
cknight@lifescicomms.com

Source: X4 Pharmaceuticals