X4 Pharmaceuticals Presents Positive Clinical Data from Phase 2 Study of Mavorixafor in WHIM Syndrome at EHA 2020

June 12, 2020

Sustained efficacy and safety trends observed for up to 28.6 months support ongoing pivotal Phase 3 trial dosing and endpoints, including selection of the measurement of time above threshold for absolute neutrophil count as primary endpoint

Significant reductions in yearly infection rate and wart burden demonstrated at 400 mg daily dose

Company to host conference call today at 8:30 a.m. ET to discuss results

CAMBRIDGE, Mass., June 12, 2020 (GLOBE NEWSWIRE) -- **X4 Pharmaceuticals, Inc.** (Nasdaq: XFOR), a leader in the discovery and development of novel therapies targeting diseases resulting from dysfunction of the CXCR4 pathway, today presented positive biomarker, efficacy, and safety data from its ongoing Phase 2 open-label extension trial of its lead candidate, mavorixafor, in patients with WHIM (warts, hypogammaglobulinemia, infections, and myelokathexis) syndrome. The results included significant reductions in yearly infection rates and wart burden in WHIM patients treated for at least six months with mavorixafor. The data were presented in an **e-poster** at the 25th European Hematology Association (**EHA**) Annual Congress, taking place virtually from June 11-14, 2020.

“We are extremely encouraged by the positive therapeutic profile of mavorixafor emerging from this study, including improvements in key biomarkers and clinical symptoms at the higher doses,” said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. “These results suggest that mavorixafor is a promising, disease-modifying therapy that, by down-regulating CXCR4/CXCL12 signaling, could lead to improved and durable clinical efficacy in patients with WHIM syndrome. We view these data as a significant de-risking event for our ongoing and pivotal Phase 3 clinical trial, from which we expect top-line data in 2022. We look forward to continuing to advance the development of mavorixafor for patients with WHIM syndrome, a rare disease that we estimate could affect more than 3,500 people in the United States and that has no currently approved treatments.”

The original Phase 2 clinical trial was an open-label, dose-escalation study that was followed by an open-label extension study to assess the safety, tolerability, dose-response, and clinical impact of mavorixafor in adult patients with genetically confirmed WHIM syndrome. The extension phase was open to patients who completed at least 24 weeks of the initial dose-escalation study and explored additional endpoints related to absolute neutrophil and lymphocyte counts, infection rates and wart burden, as well as long-term safety.

Key Data Presented:

- The Phase 2 trial results informed the design of the company’s ongoing global pivotal Phase 3 clinical

trial (**4WHIM**) in:
    - The selection of the 400 mg once-daily dose; and
    - The choice of time above threshold for absolute neutrophil counts (TAT_{ANC}), defined as the number of hours during which the absolute neutrophil count is raised above the 500 cells per microliter threshold ("time above threshold"), as the primary endpoint of the trial.
- Sustained, dose-dependent increases in WBC (white blood cells), ANC (absolute neutrophil count), and ALC (absolute lymphocyte count) were achieved; higher doses of mavorixafor were shown to increase the TAT_{ANC} at least 4.5-fold versus the TAT_{ANC} at lower doses.
- These long-term hematological improvements correlated with fewer infections and reduced numbers of cutaneous warts, two secondary clinical endpoints in the 4WHIM trial:
    - A decreased yearly infection rate from 4.63 [95%CI 3.3,6.3] events in the 12 months prior to the trial, to 2.27 [95%CI 1.4, 3.5] events when treated with mavorixafor at higher doses once daily; notably, deeper reductions in yearly infection rates correlated with increased time on treatment.
    - The patients with cutaneous warts on hands and/or feet at baseline achieved an average 75% reduction in the number of warts.
- Mavorixafor was well-tolerated for the extended duration of up to more than two years without any attributable serious adverse effects.

Poster Details:

- **Abstract #EP852:** Oral CXCR4 Antagonist Mavorixafor Treatment in Patients with WHIM Syndrome: Results of an Open-label Phase 2 Study with Long-term Extension
- **Date and Time:** Friday, June 12th, 8:30 a.m. CET / 2:30 a.m. ET

Conference Call Information:
The X4 Pharmaceuticals conference call and webcast will take place at 8:30 am ET on June 12, 2020. The conference call can be accessed by dialing (866) 721-7655 (domestic) or (409) 216-0009 (international), followed by the conference ID: 9219476. The live webcast will be accessible on the investor relations section of the company's website at **investors.x4pharma.com**. The webcast replay will be available on the website approximately two hours after the completion of the call.

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 resulting from dysfunction 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 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). X4 is continuing to leverage its insights into CXCR4 biology at its corporate headquarters in Cambridge, 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 of mavorixafor for use in WHIM, along with WHIM’s estimated prevalence. 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, the risks and uncertainties 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 7, 2020, 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.

Investors and Media:
Candice Ellis, 857-341-1043
Director, Corporate Communications & Investor Relations
Candice.Ellis@x4pharma.com

Source: X4 Pharmaceuticals