X4 Pharmaceuticals Announces Data from Phase 2 Open-Label Extension Trial of Mavorixafor in WHIM Syndrome as Published in EHA Abstract

May 14, 2020

Data reveal reductions in annual infections and warts, and sustained dose-dependent increases in neutrophil counts

These and additional Phase 2 data to be presented in an e-Poster on June 12th at the Virtual Edition of the 25th Congress of the European Hematology Association (EHA)

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 14, 2020-- X4 Pharmaceuticals, Inc. (Nasdaq: XFOR), a leader in the discovery and development of novel therapies targeting diseases resulting from dysfunction of the CXCR4 pathway, disclosed efficacy and safety data from the Phase 2 open-label extension trial of its lead candidate mavorixafor in patients with WHIM (Warts, Hypogammaglobulinemia, Infections, and Myelokathexis) syndrome. These data are included in an abstract published today and selected to be presented as an e-Poster at the 25th European Hematology Association (EHA) Annual Congress, taking place virtually from June 11-14, 2020.

The original Phase 2 clinical trial was a two-part, open-label, dose-escalation study of mavorixafor in adult patients followed by an open-label extension study to determine the safety, tolerability, and dose selection of mavorixafor in participants 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 infection rates and wart burden, along with long-term safety. The trial results informed the design of the company's ongoing pivotal Phase 3 clinical trial (4WHIM). Data from the Phase 2 trial also resulted in the receipt of Breakthrough Therapy Designation by the U.S. Food & Drug Administration for mavorixafor for the treatment of WHIM. The company recently updated its estimates regarding WHIM prevalence in the U.S. to 1,300 – 3,700 diagnosed and undiagnosed patients and expects to report top-line data from the global Phase 3 trial in 2022.

Data from patients treated with 300 mg or 400 mg once-daily doses of mavorixafor for up to 28.6 months will be presented at this year's EHA Congress. Results summarized in the abstract include:

- Observed sustained, dose-dependent increases in absolute neutrophil count (ANC) and absolute lymphocyte count (ALC).
- Long-term hematological improvements correlated with fewer infections and improved cutaneous warts.
    - Clinical yearly infection rates decreased from 4.63 in the 12 months prior to the trial, to 2.41 (a 48% reduction) at 300 mg and 2.14 (a 54% reduction) at 400 mg of mavorixafor.
    - Patients with cutaneous warts on hands and/or feet at baseline demonstrated a greater than 50% reduction in the number of warts at their last dermatological evaluation.
- Mavorixafor was well tolerated for the extended duration of more than two years without any attributable serious adverse effects.

These and additional data from the Phase 2 open-label extension study will be presented during a virtual e-Poster presentation:

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 am CEST / 2:30 am ET
Session Title: Bone marrow failure syndromes incl. PNH - Clinical

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 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 X4's plans for clinical development of mavorixafor, including the timing of completion and results of its global Phase 3 clinical trial in patients with WHIM syndrome, estimates regarding the WHIM patient population and potential market opportunity, and the potential therapeutic benefit of mavorixafor. 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.

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