X4 Pharmaceuticals Announces Positive Preliminary Data from Ongoing Phase 1b Clinical Trial of Mavorixafor in Waldenström's Macroglobulinemia as Published in EHA Abstract

May 12, 2021

Initial data from first cohort of patients indicate mavorixafor plus ibrutinib to be well tolerated, with encouraging decreases in serum IgM observed

These and additional preliminary Phase 1b data to be presented in an e-Poster on June 11 at the 2021 European Hematology Association (EHA) Annual Congress

BOSTON, Mass., May 12, 2021 (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 disclosed the first efficacy and safety data from a small cohort of patients in its ongoing Phase 1b clinical trial of its lead candidate mavorixafor in combination with ibrutinib in patients with Waldenström's macroglobulinemia. These data are included in an abstract published today and selected to be presented as an e-Poster at this year's European Hematology Association (EHA) Annual Congress, taking place virtually from June 9-17, 2021.

About the Phase 1b Clinical Trial

- The Phase 1b, open-label, multicenter, single-arm study (NCT04274738) is being conducted in the U.S., Germany, and Greece. The trial examines intra-patient dose escalation, safety, pharmacokinetics, and pharmacodynamics of mavorixafor in combination with ibrutinib in patients greater than 18 years of age with a diagnosis of Waldenström's macroglobulinemia and confirmed $MYD88^{L265P}$ and $CXCR4^{WHIM}$ genetic mutations.
- In the Phase 1 study, patients are initiated on oral doses of mavorixafor 200 mg and ibrutinib 420 mg once-daily. Mavorixafor escalation to 400 mg occurs after 28 days if no dose-limiting toxicities are observed, and to 600 mg after 400 mg is deemed safe. Patients are followed for adverse events and change from baseline in serum IgM, pharmacokinetics, and pharmacodynamic markers, including peripheral white blood cell counts (WBCs).

About Waldenström's Macroglobulinemia and Mavorixafor

- Waldenström's macroglobulinemia (WM) is a rare B-cell lymphoproliferative disorder characterized by increased Immunoglobulin M (IgM)-secreting cells and increased risk of developing symptomatic hyperviscosity syndrome. Patients with the double $MYD88^{L265P}$ and $CXCR4^{WHIM}$ mutation treated with ibrutinib (a Bruton's tyrosine kinase (BTK) inhibitor) typically experience delayed response, inferior depth of response, and/or shorter progression-free survival.
- Mavorixafor is an oral small-molecule antagonist of the CXCR4 receptor with a demonstrated ability to inhibit binding of its ligand, CXCL12; this inhibition of CXCR4 has been shown to sensitize WM cells expressing both $MYD88^{L265P}$ and $CXCR4^{WHIM}$ to BTK inhibition.

Initial Clinical Results Published (as of cut-off date of January 22, 2021)

- Of the 7 patients enrolled, all were successfully escalated from the starting dose of 200 mg mavorixafor to 400 mg, with one patient de-escalating to 200 mg due to an observed adverse event; the median drug exposure was 90 days.
- Of the total of 56 adverse events observed, none led to study discontinuation and no serious adverse events were identified.
- Of the 4 patients treated for greater than or equal to three 28-day treatment cycles, all experienced rapid and clinically meaningful decreases in IgM levels, achieving a median decrease in serum IgM of 51.0%.
    - All 4 showed an increase in peripheral WBCs.
- Six out of the 7 patients showed a decrease in IgM after 1 cycle.
- The abstract concludes that these preliminary results suggest that mavorixafor may sensitize $MYD88^{L265P}$- and $CXCR4^{WHIM}$-expressing cells to inhibition by ibrutinib in this patient population.

These and additional data from the Phase 1b clinical trial will be presented during a virtual e-Poster session:

Abstract #1424: Preliminary Clinical Data From a Phase 1b Study of Mavorixafor and Ibrutinib in Patients With Waldenström's Macroglobulinemia With MYD88 and CXCR4 Mutations

Date and Time: Friday, June 11, 9:00 am CET / 3:00 am ET
Category: Indolent and mantle-cell non-Hodgkin lymphoma - Clinical

X4 anticipates hosting an investor conference call and webcast around the time of the public release of the e-poster.

This Phase 1b clinical trial is being conducted with the support of AbbVie Inc.

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 Boston, 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 statements regarding the clinical development of mavorixafor and X4's other product candidates or programs. 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 6, 2021, 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:
Daniel Ferry
Managing Director
LifeSci Advisors
daniel@lifesciadvisors.com
(617) 430-7576

Mónica Rouco Molina
Senior Account Executive
LifeSci Communications
mroucomolina@lifescicomms.com

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