# X4 Pharmaceuticals Provides Business and Clinical Development Update

April 1, 2019

- *Expects to commence the global Phase 3 pivotal trial of mavorixafor for the treatment of patients with Warts, Hypogammaglobulinemia, Infections and Myelokathexis (WHIM) syndrome in second quarter 2019 having finalized the protocol based on FDA guidance*
- *Receives World Health Organization (WHO) approval for mavorixafor as recommended International Non-proprietary Name (INN) for X4P-001*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Apr. 1, 2019-- X4 Pharmaceuticals, Inc. (Nasdaq:XFOR), a clinical-stage biopharmaceutical company focused on the development of novel therapeutics for the treatment of rare diseases, today provided a business and clinical development update.

“This has been a transformative period for X4 with our listing on Nasdaq, finalization of our Phase 3 clinical protocol in WHIM syndrome and the recent approval from the WHO for the use of mavorixafor as our lead candidate name for X4P-001,” said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. “We look forward to advancing our rare disease pipeline throughout the year with plans to commence a global Phase 3 pivotal trial in WHIM, initiate a Phase 1 trial in severe congenital neutropenia and a Phase 1/2 trial in Waldenström macroglobulinemia, and to disclose data from our ongoing Phase 2a trial in clear cell renal cell carcinoma at a medical meeting later this year.”

**Business Update**

In March 2019, X4 Pharmaceuticals commenced trading on the Nasdaq Capital Market under the symbol “XFOR.”

In March 2019, X4 submitted its orphan drug designation request to the European Medicines Agency (EMA) for mavorixafor for the treatment of WHIM syndrome. In October 2018, X4 received orphan drug designation from the FDA for the treatment of WHIM syndrome.

X4 also recently received approval from the World Health Organization (“WHO”) for mavorixafor as the recommended International Non-proprietary Name (“INN”) for the company’s lead drug candidate, X4P-001. Mavorixafor is a first-in-class, oral, allosteric antagonist of the chemokine receptor CXCR4 with a demonstrated 23-hour half-life and bioavailability profile that support once-daily oral dosing.

**Clinical Development Update**

X4 has finalized the clinical trial protocol based on guidance from the FDA for its randomized, placebo controlled double blinded Phase 3 pivotal trial of mavorixafor for the treatment of patients with WHIM syndrome and expects to commence the study in the second quarter 2019. As reviewed with the FDA, the primary endpoint will be the biomarker of neutrophil count time above threshold (‘TAT’) where the threshold is defined as 500 cells/uL. The Phase 3 pivotal trial’s secondary endpoints, including infection rates and wart burden assessments, and secondary endpoint hierarchy was also reviewed with the FDA. All enrolled patients ages 12 years and older will receive 400 mg, once daily, of mavorixafor. The Phase 3 pivotal trial will enroll patients from the US and from other global sites.

X4 continues to conduct the Phase 2 open label extension study following the completion of the dose titration portion of the Phase 2 trial for the treatment of patients with WHIM syndrome in March 2018. Five patients are continuing to receive mavorixafor in the Phase 2 open-label extension study and the company plans to provide future updates on the extension study.

In March 2019, the FDA included WHIM syndrome in a guidance for industry for Severely Debilitating or Life-Threatening Hematologic Disorders. X4 has made progress in educating key stakeholders regarding the clinical impact of WHIM syndrome.

X4 is on track to commence a Phase 1 clinical trial of mavorixafor for the treatment of patients with severe congenital neutropenia (SCN) in the United States in 2019. The trial is designed to determine the genetic profile of SCN patients and assess their pharmacodynamic response to mavorixafor.

X4 also plans to commence a multi-national Phase 1/2 clinical trial of mavorixafor in combination with ibrutinib for the treatment of patients with Waldenström macroglobulinemia in 2019. The study population will focus on patients with WHIM-like mutations in CXCR4 who are known to respond poorly to standard of care.

X4 has completed enrollment in the open label Phase 2a portion of its ongoing Phase 1/2 clinical trial of mavorixafor in combination with axitinib in clear cell renal cell carcinoma (ccRCC) patients. The Company plans to unveil progression-free survival (PFS) data as part of an anticipated abstract to be submitted for presentation at a major medical conference in the second half of 2019.

**About X4 Pharmaceuticals**
X4 Pharmaceuticals is developing novel therapeutics designed to improve immune cell trafficking to treat rare diseases, including primary immunodeficiencies and cancer. X4’s oral small molecule drug candidates antagonize the CXCR4 pathway, which plays a central role in immune surveillance. X4’s most advanced product candidate, mavorixafor (X4P-001), will be commencing a global Phase 3 pivotal trial in patients with WHIM syndrome, a rare genetic, primary immunodeficiency disease, in the second quarter of 2019 and is currently also under investigation in a Phase 2a

clinical trial in clear cell renal cell carcinoma. X4 was founded and is led by a team with extensive product development and commercialization expertise, including several former members of the Genzyme leadership team, and is located in Cambridge, Massachusetts. For more information, visit www.x4pharma.com.

**Forward-Looking Statements**
This press release contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statement of historical facts, included in this press release regarding our strategy, future operations, and plans are forward-looking statements. Examples of such statements include, but are not limited to, statements relating to plans for, or progress, scope, cost, duration or results or timing for the initiation, completion or availability of results of development of mavorixafor (X4P-001) or any of our other product candidates or programs, including regarding the Phase 3 clinical trial of mavorixafor for the treatment of patients with WHIM syndrome, the target indication(s) for development, the size, design, population, location, conduct, objective, duration or endpoints of any clinical trial, or the timing for initiation or completion of or reporting of results from any clinical trial, the potential benefits of mavorixafor, or any other product candidate or program or the commercial opportunity in any target indication; or the potential benefits of orphan drug designation. Actual results or events could differ materially from the plans, intentions, expectations and projections disclosed in the forward-looking statements. Various important factors could cause actual results or events to differ materially from the forward-looking statements that X4 makes, including, but not limited to, the risk that trials and studies may be delayed and may not have satisfactory outcomes, potential adverse effects arising from the testing or use of mavorixafor or other product candidates, the risk that costs required to develop mavorixafor or other product candidates or to expand our operations will be higher than anticipated and other risks described in the “Risk Factors” section of the Registration Statement on Form S-4 filed by X4 Pharmaceuticals with the SEC and declared effective by the SEC on February 14, 2019. X4 does not assume any obligation to update any forward-looking statements, except as required by law.

View source version on businesswire.com: https://www.businesswire.com/news/home/20190401005255/en/

Source: X4 Pharmaceuticals, Inc.

**Investors:**
Stephanie Carrington
Westwicke, an ICR company
646-277-1282
Stephanie.Carrington@icrinc.com

**Media:**
Darcie Robinson
Westwicke, an ICR company
203-919-7905
Darcie.Robinson@icrinc.com