X4 Pharmaceuticals to Present Phase 2a Data for Mavorixafor in Combination with Axitinib at European Society for Medical Oncology (ESMO) 2019 Congress

September 12, 2019

Management to host investor conference call and webcast on Monday, September 30 at 8:00 AM EDT / 2:00 PM CEST

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 12, 2019-- X4 Pharmaceuticals, Inc. (Nasdaq: XFOR), a clinical stage biotechnology company focused on the development of novel therapeutics for the treatment of rare diseases, today announced it will present clinical data on its lead product candidate, mavorixafor (X4P-001), in combination with Inlyta® (axitinib) at the upcoming European Society for Medical Oncology (ESMO) 2019 Congress, taking place September 27 – October 1 in Barcelona, Spain. The presentation will detail final safety and efficacy results from the Company's Phase 2a portion of an open-label Phase 1/2 clinical trial of mavorixafor in combination with axitinib in patients with advanced clear cell renal cell carcinoma (ccRCC).

Details of the presentation are as follows:

Abstract #2521: Safety and Efficacy of the Oral CXCR4 Inhibitor X4P-001 + Axitinib in Advanced Renal Cell Carcinoma Patients: An Analysis of Subgroup Responses by Prior Treatment
Date and Time: Monday, September 30, 2019; 8:45 – 9:45 AM CEST
Session Type: Poster Discussion Session, Discussion 2 – Immunotherapy of Cancer
Presentation #: 1186PD
Location: Malaga Auditorium, Hall 5

Details of the investor conference call and webcast are as follows:

Time and Date: Monday, September 30 at 8:00 AM EDT / 2:00 PM CEST
US Toll-Free Dial-In Number: (866) 721-7655
International Dial-In Number: (409) 216-0009 / Spain 0934923253
Conference ID: 4787329
Webcast: A live audio webcast of the conference call may be accessed in the "Investors" section of the Company's website at the following link.

About Mavorixafor

X4 Pharmaceuticals' lead product candidate, mavorixafor (X4P-001), is a potential first-in-class, once-daily, oral inhibitor of CXCR4, currently in Phase 3 development for the treatment of WHIM syndrome, a rare, inherited, primary immunodeficiency disease caused by genetic mutations in the CXCR4 receptor gene. Mavorixafor has demonstrated proof of concept in WHIM syndrome in a Phase 2 trial, including clinically meaningful increases in neutrophil and lymphocyte biomarker counts, as well as a trend of reduction in infection rates and wart burden, and a favorable safety profile. Mavorixafor was designated orphan drug status by the U.S. Food and Drug Administration in 2018 and by the European Commission in 2019 for the treatment of WHIM syndrome, and is also in development for the treatment of Severe Congenital Neutropenia (SCN), Waldenström's macroglobulinemia (WM), and clear cell renal cell carcinoma (ccRCC).

About X4 Pharmaceuticals

X4 Pharmaceuticals is developing novel therapeutics designed to improve immune cell trafficking to treat rare diseases, including primary immunodeficiencies and certain cancers. The company'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), is in a global Phase 3 pivotal trial in patients with WHIM syndrome, a rare, inherited, primary immunodeficiency disease, and is currently also under investigation in combination with axitinib in the Phase 2a portion of an open-label Phase 1/2 clinical trial in clear cell renal cell carcinoma (ccRCC). X4 is also planning to commence clinical trials of mavorixafor in Severe Congenital Neutropenia (SCN) and Waldenström's macroglobulinemia (WM) in 2019. The company was founded and is led by a team with extensive biopharmaceutical product development and commercialization expertise and is committed to advancing the development of innovative medicines on behalf of patients with limited treatment options. X4 is a global company that is headquartered in Cambridge, Massachusetts with research offices based in Vienna, Austria. 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. The words "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Forward-looking statements include, but are not limited to, statements regarding the reporting of data from X4's Phase 2a portion of the Phase 1/2 clinical trial of mavorixafor in combination with axitinib in advanced clear cell renal cell carcinoma (ccRCC), X4's business strategy, X4's plans to develop and commercialize its product candidates and the safety and efficacy of X4's product candidates. These statements are subject to various risks and uncertainties, actual results could differ materially from those projected and X4 cautions investors not to place undue reliance on the

forward-looking statements in this press release. These risks and uncertainties include, without limitation, 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. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, the risks and uncertainties described in the section entitled “Risk Factors” in X4’s most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC), as updated by X4’s Current Report on Form 8-K filed with the SEC on April 11, 2019, 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 subsequently occurring events or circumstances.

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