X4 Pharmaceuticals Granted New Composition of Matter Patent for Late-Stage Clinical Candidate Mavorixafor

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– Patent expected to provide exclusivity through 2038 –

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 12, 2020-- X4 Pharmaceuticals, Inc. (Nasdaq: XFOR), a clinical-stage biopharmaceutical company focused on the development of novel therapeutics for the treatment of rare diseases, today announced that the United States Patent and Trademark Office (USPTO) has issued United States Patent No. 10,548,889, which is expected to provide exclusivity of X4's lead therapeutic candidate, mavorixafor (X4P-001), through 2038.

"This new patent which covers critical compositions of matter enhances our already robust patent portfolio, further strengthening and extending the potential commercial horizon for mavorixafor," said Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "We expect that this long patent runway will assist us in maximizing the full therapeutic potential of mavorixafor, which we are currently investigating across a number of rare disease indications."

About Mavorixafor

X4 Pharmaceuticals' lead product candidate, mavorixafor (X4P-001), is a potential first-in-class, once-daily, oral inhibitor of CXCR4, currently in a Phase 3 clinical trial 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 clinical 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 recently granted Breakthrough Therapy Designation by the U.S. Food and Drug Administration (FDA) for the treatment of adult patients with WHIM syndrome, and was granted orphan drug status by the FDA in 2018 and by the European Commission in 2019 for the treatment of WHIM syndrome. Mavorixafor is also being developed by X4 to treat 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 an open-label Phase 1/2 clinical trial in clear cell renal cell carcinoma (ccRCC), with several patients remaining on therapy over 12 months beyond the primary endpoint. X4 is further investigating mavorixafor in Phase 1b clinical trials for the treatment of Severe Congenital Neutropenia (SCN), and with ibrutinib for the treatment of Waldenström's macroglobulinemia (WM). X4 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. 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 and X4's exclusive rights over intellectual property. These statements are subject to various risks and uncertainties including, 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 product candidates or to expand X4's operations will be higher than anticipated, and the risks and uncertainties arising from intellectual property claims and/or challenges. 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 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 new events or circumstances, except as required by law.

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