X4 Pharmaceuticals and Abbisko Therapeutics Enter into Oncology Development and Commercialization Agreement for Mavorixafor in Greater China

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Cambridge, MA and Shanghai — July 17, 2019—X4 Pharmaceuticals, Inc. (Nasdaq: XFOR), a clinical-stage biopharmaceutical company focused on the development of novel therapeutics for the treatment of rare diseases, and Abbisko Therapeutics, a company with an extensive pipeline of targeted therapeutics with first-in-class or best-in-class potential, today announced they have entered into an agreement to develop and commercialize X4's product candidate, mavorixafor, in combination with checkpoint inhibitors or other agents in Greater China for oncology indications. Mavorixafor is a potentially first-in-class, once-daily, oral, small molecule antagonist of chemokine receptor CXCR4.

This agreement provides Abbisko with the exclusive rights in China, Taiwan, Hong Kong and Macau to develop and commercialize maxorixafor in combination with checkpoint inhibitors or other agents in oncology indications – including pancreatic cancer, ovarian cancer and triple negative breast cancer, which will be explored initially. X4 retains full rest-of-world rights to develop and commercialize mavorixafor outside of Greater China for all indications, and the ability to utilize any data generated pursuant to the Abbisko collaboration for rest-of-world development.

"We are pleased to enter into this strategic partnership with the experienced team at Abbisko. This collaboration enables us to leverage Abbisko's research and development expertise to explore mavorixafor's potential benefit in advanced cancer patients and to potentially capture value from the growing oncology markets in Greater China, while enabling X4 to maintain its focus on the company's ongoing rare disease programs," commented Paula Ragan, Ph.D., President and Chief Executive Officer of X4 Pharmaceuticals. "Abbisko's top-notch investor syndicate, strong leadership with global oncology R&D experience, and broad healthcare network in Greater China provide a complementary opportunity to expand mavorixafor's potential for patients into solid tumor oncology indications, which represent areas of significant unmet need."

"Mavorixafor has demonstrated proof of concept and a favorable safety profile in a Phase 2 trial in patients with WHIM syndrome, which is caused by compromised immune cell trafficking. We look forward to further realizing mavorixafor's potential in broad oncology indications in combination with immune checkpoint inhibitors and other therapies, for the benefit of patients with significant unmet medical needs," said Dr. Yaochang Xu, Chief Executive Officer of Abbisko Therapeutics. "Targeting CXCR4 has strong mechanistic rationales in oncology and we believe mavorixafor will bring transformative value to Abbisko's portfolio with clear synergies with our internal pipeline programs."

About Mavorixafor
X4 Pharmaceutical's lead product candidate, mavorixafor (X4P-001), is a potentially 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 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 for the treatment of WHIM and is also in development for 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.

About Abbisko Therapeutics
Founded in April 2016, Abbisko Therapeutics Co., Ltd. is a biopharmaceutical company dedicated to discovering and developing innovative therapeutics to treat cancer and other diseases with unmet medical needs. The founders and leadership team of Abbisko are a group of industrial veterans with strong leadership and managerial experiences from top international pharmaceutical companies. They have led and participated in the discovery and development of multiple FDA-approved drugs. In three years, Abbisko has established a strong oncology pipeline with multiple programs entering the clinic.

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 statements regarding 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 Phase 2a portion of the Phase 1/2 clinical trial of mavorixafor in combination with axitinib in ccRCC, or plans to commence clinical trials of mavorixafor in SCN and WM, 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, as well as the expected benefits of the Abbisko relationship to X4 or Abbisko. 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 and the risk that X4's relationship with Abbisko will not be successful. 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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