# X4 Pharmaceuticals and The Leukemia & Lymphoma Society Announce Collaboration to Advance Development of Mavorixafor in Waldenström's Macroglobulinemia

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*Mavorixafor selected for LLS's Therapy Acceleration Program ® (TAP)*

CAMBRIDGE, Mass. & RYE BROOK, N.Y.--(BUSINESS WIRE)--May 15, 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 The Leukemia & Lymphoma Society (LLS) today announced a collaboration to accelerate the development of X4's lead product candidate, mavorixafor (X4P-001) for the treatment of Waldenström's macroglobulinemia (WM), a rare form of non-Hodgkin lymphoma.

Mavorixafor was selected for LLS's Therapy Acceleration Program ® (TAP), a strategic initiative where LLS builds business alliances and collaborations with biotechnology companies and academic researchers to speed the development of new therapies for blood cancers. Under the collaboration, X4 will conduct a multi-national Phase 1/2 clinical trial to evaluate the safety and assess the preliminary anti-tumor activity of mavorixafor in combination with ibrutinib in WM patients. The trial is planned to commence this year. Lee Greenberger, Ph.D., chief scientific officer of LLS, will also serve as a member of an advisory board to X4, providing important strategy and partnership guidance throughout the trial.

"LLS's selection of mavorixafor for TAP collaboration and investment reinforces its potential as a novel therapy for Waldenström's macroglobulinemia. Approximately 30 to 40 percent of WM patients have a CXCR4 mutation, and a number of these patients do not respond well to current therapies," said Paula Ragan, Ph.D., president and chief executive officer of X4 Pharmaceuticals. "We look forward to working closely with Dr. Greenberger and the LLS TAP team to gain valuable data and insights throughout the upcoming clinical trial as we work to bring a new therapeutic option to patients with this rare form of cancer."

Mavorixafor is a first-in-class, oral, small molecule allosteric antagonist of the chemokine receptor CXCR4 and is designed to address certain rare primary immunodeficiency diseases and certain cancers, including lymphomas, in which genetic mutations in CXCR4 create abnormal trafficking of white blood cells and play a role in disease process.

"Through TAP, LLS is committed to advancing the development of promising investigational therapies that we believe have potential to improve standards of care for patients, especially in disease areas with high unmet medical need, such as Waldenström's macroglobulinemia," said Dr. Greenberger. "Mavorixafor has demonstrated early promise in other disease areas with CXCR4 mutations, including solid tumors, and its potential application among CXCR4-mutant WM patients makes it an excellent fit and an important asset within our program as we work with innovative companies like X4 to uncover and develop cutting-edge therapies for patients with blood cancers."

**About Waldenström's Macroglobulinemia**
Waldenström's macroglobulinemia (WM) is a rare form of non-Hodgkin lymphoma and B-cell lymphoproliferative disorder. According to the American Cancer Society, approximately three per one million people are diagnosed each year, including 1,400 new cases in the United States annually. Recent advancements in whole-genome sequencing have identified genetic mutations in the disease similar to WHIM syndrome, a rare congenital primary immunodeficiency characterized by warts, hypogammaglobulinemia, infection and myelokathexis. Approximately 30 to 40 percent of WM cases express mutations in the CXCR4 gene in the cancer cells. In WM, somatic mutations of CXCR4 are associated with active tumor cells and possible drug resistance, including resistance to anti-CD20 monoclonal antibodies and Burton tyrosine kinase (BTK) inhibitors, such as ibrutinib, the current standard of care. WM patients with this somatic mutation have a dramatically reduced median progression-free survival, or mPFS, of approximately two years, whereas patients without the mutation have a mPFS of well over five years.

**About the Therapy Acceleration Program®**
The Leukemia & Lymphoma Society's Therapy Acceleration Program ® (TAP) identifies and funds innovative projects related to therapies, supportive care or diagnostics that have the potential to change the standard of care for patients with blood cancer, especially in areas of high unmet medical need. TAP funding assists both clinical investigators and companies in gaining critical clinical proof of concept data that better enables them to obtain the resources they need or a partner to complete the testing, registration and marketing of new treatments, supportive care and diagnostics for leukemia, lymphoma and myeloma. TAP funding is different from the traditional grant at LLS. The TAP review process is separate from the grant process and LLS's TAP staff play an active advisory role and closely monitor each approved project. To learn more about how TAP works, please click here.

**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. 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), is expected to commence 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.

**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 and the Phase 1/2 clinical trial of mavorixafor for the treatment of patients with 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. 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 the collaboration with LLS will not be successful, and 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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