

X4 Pharmaceuticals to Present Clinical Data from Phase 2 Expansion Study of Combination of X4P-001-IO and Inlyta[®] (axitinib) in Patients with Clear Cell Renal Cell Carcinoma

Study designed to evaluate X4P-001-IO in combination therapy to be presented at the Annual Meeting of the Association Society of Clinical Oncology

Cambridge, MA – May 16, 2018 – X4 Pharmaceuticals, a clinical stage biotechnology company developing novel CXCR4 inhibitor drugs to improve immune cell trafficking to treat cancer and rare disease, today announced that an abstract highlighting X4P-001-IO, the company's CXCR4 antagonist, has been selected for poster presentation at the 2018 Association Society of Clinical Oncology (ASCO) annual meeting, taking place June 1-5 in Chicago. The presentation will describe clinical results from the Phase 2 expansion of an ongoing Phase 1/2 study of X4P-001-IO in combination with Inlyta[®] (axitinib) in patients with clear cell renal cell carcinoma (ccRCC).

Details of the presentation on X4P-001 are as follows:

- Title:** A phase 1/2 study evaluating the efficacy and safety of the oral CXCR4 inhibitor X4P-001 in combination with axitinib in patients with advanced renal cell carcinoma
- Authors:** Ulka Vaishampayan, M.D., Barbara Ann Karmanos Cancer Institute, Detroit, MI
Michael Atkins, M.D., Georgetown-Lombardi Comprehensive Cancer Center, Washington, DC
- Abstract #:** 4510
- Poster Session:** Genitourinary (Nonprostate) Cancer
- Session Type:** Poster Discussion Session, chosen as a select poster where expert discussants will highlight the most clinically applicable and novel posters, with abstract authors participating as panel members
- Date and Time:** June 2, 2018, 1:15 PM-2:30 p.m. CT

About X4P-001-IO in Cancer

X4P-001-IO is an investigational selective, oral, small molecule antagonist of C-X-C receptor type 4 (CXCR4). CXCR4 is a chemokine receptor present in abundance on certain immune cells and cancer cells and it plays a critical role in immune cell trafficking, infiltration and activation in the tumor microenvironment. CXCR4 signaling is disrupted in a broad range of cancers, facilitating tumor growth by allowing cancer cells to evade immune detection and creating a pro-tumor microenvironment. X4P-001-IO has the ability to help restore immunity within the tumor microenvironment and has the potential to enhance the anti-tumor activity of approved and emerging oncology agents, such as checkpoint inhibitors and targeted therapies. X4P-001-IO is being investigated in several clinical studies in solid tumors.

About X4 Pharmaceuticals

X4 Pharmaceuticals is developing novel therapeutics designed to improve immune cell trafficking to treat cancer and rare diseases. 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, X4P-001-RD, is in a Phase 2/3 study in patients with WHIM syndrome, a rare genetic, primary immunodeficiency disease. X4P-001-IO is currently under investigation in multiple clinical studies in solid tumors. X4 was founded and is led by a team with deep product development and commercialization

expertise, including several former members of the Genzyme leadership team, and is located in Cambridge, MA. For more information, visit www.x4pharma.com.

Inlyta[®] is a registered trademark of Pfizer.

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