

X4 Pharmaceuticals to Present Clinical Data from Pilot Study of Combination of X4P-001-IO and Opdivo® (nivolumab)

May 8, 2018

Study designed to evaluate X4P-001-IO in expanded range of combination treatment settings to be presented at the Annual Meeting of the Association for Cancer Immunotherapy

Cambridge, MA - May 8, 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 and oral presentation at the 16th Annual Meeting of the Association for Cancer Immunotherapy (CIMT), taking place May 15-17 in Mainz, Germany. The presentations will describe clinical results from a pilot study of X4P-001-IO in combination with Opdivo® (nivolumab) in patients with clear cell renal cell carcinoma (ccRCC), expanding the range of combination treatment settings in which X4P-001-IO has been studied.

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

Title: The safety, tolerability, and preliminary clinical activity of the CXCR4 inhibitor X4P-001 and nivolumab in renal cell carcinoma patients that are refractory to nivolumab monotherapy

Author: David F. McDermott, M.D., Beth Israel Deaconess Medical Center, Harvard Medical School

Abstract #: 77

Poster Session: Tumor Biology and Interaction with the Immune System, May 15th, 3:00 – 5:00 PM CET

Oral Presentation Session: Clinical Trials, May 17th, 11:45 AM - 1:00 PM CET

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 x4.thevatesnetwork.com.

Opdivo® is a registered trademark of Bristol-Myers Squibb Company.

Media Contact:

Kathrvn Morris Tel: 914-204-6412

kathrvn@thevatesnetwork.com