

X4 Pharmaceuticals to Report Clinical Biomarker Data with X4P-001 at the Upcoming 2018 American Association for Cancer Research (AACR) Annual Meeting

March 15, 2018

Cambridge, MA – March 15, 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, the Company's CXCR4 antagonist, has been selected for presentation at the 2018 American Association for Cancer Research (AACR) Annual Meeting, taking place April 14-18, 2018 in Chicago, IL.

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

Poster Title: X4P-001, an oral bioavailable CXCR4 antagonist, enhances immune cell infiltration and activation in the tumor microenvironment of melanoma

Author: Robert H.I. Andtbacka, Huntsman Cancer Institute, University of Utah

Session: Immune Response to Therapies 1

Abstract #: 613

Date & Time: Sunday April 15, 2018 1:00 PM - 5:00 PM CT

"We are pleased to present these clinical biomarker data from our ongoing Phase 1b clinical study with X4P-001 in patients with melanoma," said Sudha Parasuraman, M.D., Chief Medical Officer of X4 Pharmaceuticals. "These results demonstrate enhanced immune cell activation and lymphocyte infiltration with X4P-001, alone and in combination with the anti-PD-1 immunotherapy pembrolizumab (Keytruda®), in the melanoma tumor microenvironment."

About X4P-001-IO in Cancer

X4P-001-IO is an investigational selective, oral, small molecule inhibitor of CXCR4 (C-X-C receptor type 4) that regulates the tumor microenvironment thereby enhancing endogenous anti-tumor responses. CXCR4 is a chemokine receptor that modulates immune function and angiogenesis through the trafficking of key immune cells such as T- cells, dendritic cells, and myeloid derived suppressor cells. 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 is being investigated in three separate 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 inhibit the CXCR4 receptor, a 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 Phase 1/2 studies in refractory clear cell renal cell carcinoma (ccRCC) and melanoma. 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 please visit X4pharma.com.