First Data from Combination of X4P-001-IO and Inlyta® (axitinib) in Patients with Clear Cell Renal Cell Carcinoma Will Be Presented at the European Society of Medical Oncology 2017 Congress

August 31, 2017

CAMBRIDGE, Mass., August 31, 2017 – X4 Pharmaceuticals, a clinical stage biotechnology company developing novel CXCR4 inhibitor drugs to improve immune cell trafficking to treat cancer and rare diseases, today announced that the European Society of Medical Oncology has published Phase 1 data from an ongoing Phase 1/2 study of X4P-001-IO in combination with Inlyta® (axitinib), Pfizer's VEGFR kinase inhibitor. The safety and tolerability of the combination along with preliminary efficacy data will be highlighted in a poster presentation at the European Society of Medical Oncology 2017 Congress, taking place September 8-12 in Madrid, Spain.

"These data from our Phase 1/2 trial evaluating X4P-001-IO plus Inlyta ® in ccRCC contribute to our knowledge of the therapeutic potential of CXCR4 inhibition when combined with VEGFR inhibition," said Sudha Parasurman, MD, Chief Medical Officer of X4. "These results demonstrate that the combination is well tolerated and demonstrates preliminary signs of clinical activity in a heavily pre-treated ccRCC patient population. We look forward to sharing updated data as our trial progresses."

Details of the Poster Presentations on X4P-001-IO:

Poster Title: A Phase 1 dose finding study of X4P-001 (an oral CXCR4 inhibitor) and axitinib in patients with advanced renal cell carcinoma (RCC)
Author: McDermott, David
Abstract #: 896P
Session: Genitourinary Tumors, Non-Prostate
Date & Time: Sunday, September 10 1:15 PM – 2:15 PM
Location: Hall 8

About X4P-001-IO in Cancer

X4P-001-IO is currently in Phase 1/2 testing in refractory clear cell renal cell carcinoma (ccRCC) and other solid tumor indications. Based on promising preclinical studies, X4P-001 is being evaluated in clinical studies in combination with approved cancer therapies, including tyrosine kinase inhibitors and checkpoint inhibitors. X4P-001-IO is an oral, small molecule inhibitor of CXCR4, or C-X-C receptor type 4, the receptor for the chemokine CXCL12. Recent studies demonstrate that CXCR4/CXCL12 is a primary receptor-ligand pair that cancer cells and surrounding stromal cells use to block normal immune function and promote angiogenesis through the trafficking of T-effector and T-regulatory cells, as well as myeloid derived suppressor cells (MDSCs), in the tumor microenvironment.[1,2]

About Renal Cell Carcinoma

Kidney cancer is among the ten most common cancers in both men and women with more than 60,000 new diagnoses each year in the United States.[3] Clear cell renal cell carcinoma (ccRCC) is the most common form of kidney cancer, and advanced ccRCC accounts for approximately 20% of the patient population. Therapies for advanced ccRCC include immunotherapies, mammalian target of rapamycin (mTOR) kinase inhibitors, and angiogenesis inhibitors, such as vascular endothelial growth factor (VEGF) inhibitors.[4] There continue to be unmet medical needs with advanced ccRCC because durable responses remain a serious clinical challenge for patients with advanced disease.

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.

[1] Feig C., et. al., "Targeting CXCL12 from FAP-expressing carcinoma-associated fibroblasts synergizes with anti-PD-L1 immunotherapy in pancreatic cancer," PNAS, Oct. 31, 2013.

[2] Guo F., et. al., "CXCL12/CXCR4: a symbiotic bridge linking cancer cells and their stromal neighbors to oncogenic communication networks," Oncogene, May 11, 2015.

[3] National Cancer Institute, "Surveillance, Epidemiology, and End Results Program," http://seer.cancer.gov/statfacts/html/kidrp.html

[4] Kidney Cancer Association, "Therapies for Advanced Kidney Cancer,"

http://www.kidneycancer.org/knowledge/learn/therapies-for-advanced-kidney-cancer/