X4 Pharmaceuticals Announces Initiation of the Phase 2 Expansion of its Phase 1/2 Study of X4P-001 in Patients with Advanced Clear Cell Renal Cell Carcinoma

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Phase 2 dose of X4P-001 in combination with Inlyta® selected from ongoing Phase 1/2 study

CAMBRIDGE, Mass., May 25, 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 dosing of the first patient in the Phase 2 portion of the ongoing Phase 1/2 study evaluating X4P-001, the company's lead CXCR4 inhibitor, in combination with Inlyta ® (axitinib) in patients with advanced clear cell renal cell carcinoma (ccRCC). Full results from the Phase 1 portion of the study including the selection of the once daily oral dose used in the expansion cohort will be presented at an upcoming medical meeting.

In addition to safety and tolerability, the Phase 2 portion of the study will evaluate clinical efficacy as measured by objective response rate (ORR), duration of response (DOR), and progression free survival (PFS) and will explore the correlation of biomarkers with efficacy. Multiple cancer centers with leading renal cell carcinoma researchers in the U.S. and South Korea are participating in the study.

"Efficiently progressing this study into Phase 2 is an important milestone for the development of X4P-001 and the CXCR4-targeted therapeutic approach," said Sudha Parasuraman, MD, Chief Medical Officer of X4. "Having established the Phase 2 combination dose of X4P-001, we are now focused on augmenting proof of concept data for this critically important biological axis known to play a key role in immune cell trafficking."

In addition to this Phase 1/2 study of X4P-001 in combination with Inlyta®, a VEGF kinase inhibitor, X4 has additional oncology clinical studies ongoing, including a Phase 1/2 study in patients with advanced ccRCC to evaluate X4P-001 in combination with Opdivo® (nivolumab), and a Phase 1b biomarker study in patients with advanced melanoma to evaluate X4P-001 in combination with Keytruda® (pembrolizumab).

About X4P-001 in Cancer

X4P-001, the company's lead drug candidate, 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 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 lead drug candidate (X4P-001) is currently in Phase 2/3 clinical development with the formulation X4P-001-RD for the treatment of patients with a rare, genetic primary immunodeficiency disease, WHIM syndrome, and is in Phase 1/2 clinical development as a potential cancer therapy, for the treatment of patients with refractory clear cell renal cell carcinoma (ccRCC) and other solid tumor indications. 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/