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

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CAMBRIDGE, Mass., May 9, 2016 – X4 Pharmaceuticals, a clinical stage biotechnology company developing novel CXCR4 inhibitor drugs to improve immune cell trafficking and increase the ability for T-cells to track and destroy cancer, today announced dosing of the first patient in a Phase 1/2 study of X4P-001, the Company's lead CXCR4 inhibitor, in patients with advanced clear cell renal cell carcinoma (ccRCC).

The Phase 1 portion of the study will test the safety and tolerability of escalating doses of X4P-001 in combination with Inlyta® (axitinib), a kinase inhibitor approved for the treatment of advanced RCC after failure of one prior systemic therapy. The study is designed to establish a maximum tolerated dose (MTD), or a recommended dose if the MTD is not achieved, for the drug combination. Preliminary results from the Phase 1 portion of the study are expected in early 2017, followed by the initiation of the Phase 2 portion of the study. Multiple U.S. cancer centers with leading renal cell carcinoma researchers will participate in the study. Pfizer is providing axitinib for use in the study.

"Many ccRCC patients do not achieve durable responses when treated with the currently approved targeted therapies or immunotherapies" said David McDermott, M.D, study investigator and lead of the Dana Farber/Harvard Cancer Center Kidney Cancer Program. "Based on the pre-clinical evidence in models of kidney cancer and a safety profile generated in prior clinical studies, X4P-001 has the potential to provide a meaningful new treatment option for ccRCC patients in combination with axitinib."

"Since our recent Series A financing, our team at X4 has rapidly executed on our clinical plan to initiate the study of X4P-001 as a potential treatment for patients with advanced ccRCC, a cancer with serious unmet needs." said Paula Ragan, PhD, President and CEO of X4. "Given the growing evidence that CXCR4 inhibition plays an important role modulating the tumor microenvironment, we see this as the beginning of multiple opportunities that we will pursue with our portfolio of CXCR4 inhibitors."

About X4P-001

X4P-001 is an oral, small molecule inhibitor of CXCR4, or C-X-C receptor type 4, the receptor for the chemokine CXCL12 (also known as stromal derived factor-1, or SDF-1). 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] Pre-clinical studies have demonstrated X4P-001 activity alone and in combination with approved cancer therapies including tyrosine kinase inhibitors and checkpoint inhibitors resulted in an increased tumor-specific immune response and significant delays in tumor growth. X4P-001 was previously tested in over 70 subjects in four prior clinical trials in healthy volunteers and HIV-infected patients and was shown to be safe and well tolerated.

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 and increase the ability for T-cells to track and destroy cancer cells. The company's oral small molecule drug candidates inhibit the CXCR4 receptor, a pathway which plays a central role in promoting the immunosuppressive and pro-angiogenic microenvironment of many cancers. X4P-001, the company's lead program, is open for patient recruitment to enter the Phase 1/2 study in patients with advanced clear cell renal cell carcinoma (ccRCC) and planned for other solid tumor indications, and its second program, X4P-002, is in pre-clinical development. 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/