Clinical Data for X4P-001-IO in Combination with Inlyta® (Axitinib) Demonstrated Encouraging Overall Response Rates (including a Complete Response) and Disease Control Rates in Patients with Clear Cell Renal Cell Carcinoma

Phase 1 dose escalation completed and enrollment in Phase 2 expansion continues

CAMBRIDGE, Mass., October 30, 2017 – X4 Pharmaceuticals, a clinical stage biotechnology company developing a novel CXCR4 inhibitor to improve immune cell trafficking to treat cancer and rare diseases, today announced updated results from the Phase 1 part of an ongoing Phase 1/2 study of X4P-001-IO in combination with Inlyta® (axitinib) in patients with clear cell renal cell carcinoma (ccRCC).

The results in patients with ccRCC who received the combination treatment of X4P-001-IO, a CXCR4 inhibitor, and Inlyta, Pfizer's VEGFR kinase inhibitor, showed an objective response rate (ORR) of 29%, including 1 patient achieving a confirmed complete response (CR), with an encouraging disease control rate (DCR) of 93%. 31% of patients entering the study had received one prior line of therapy while the majority of patients (69%) had received at least two prior lines of therapy. The data were presented at the 2017 AACR-NCI-EORTC Molecular Targets and Cancer Therapeutics Conference on October 29 in Philadelphia.

"The combination of CXCR4 inhibition and VEGFR inhibition shows promising clinical results in this very difficult to treat population of patients with ccRCC. These results suggest that X4P-001-IO may address some of the limitations and augment the clinical utility of axitinib, which is a clinically meaningful drug in the treatment of patients with advanced metastatic ccRCC," said Michael Atkins, MD, Deputy Director, Georgetown Lombardi Comprehensive Cancer Center, William M. Scholl Professor of Oncology at Georgetown University School of Medicine, and lead investigator of the study. "These results, while early, are very promising with a strong disease control rate and a manageable safety profile."

Results from the 16 patients with advanced ccRCC enrolled in the dose escalation part of the ongoing Phase 1/2 study as of the data cutoff date of October 2, 2017 were presented and highlights of the poster presentation include:

- The combination of X4P-001-IO and Inlyta showed one confirmed complete response (CR) and produced a DCR and ORR of 93 percent (13/14) and 29 percent (4/14), respectively, in the evaluable patient population.
- The median duration on treatment at the data cutoff was 22.1 weeks and 44 percent of patients had been exposed to study treatment for at least 24 weeks.
- X4P-001-IO in combination with Inlyta was considered to be safe and generally well tolerated. The most frequent treatment-related adverse events (AEs) were hypertension, diarrhea, fatigue, nausea, decreased appetite, headache and dry eye. No grade 4 or 5 AEs were observed.
- Pharmacodynamic (PD) measurements demonstrated that the 400 mg dose inhibited the intended target chemokine receptor CXCR4.
- Based on the study results, a dose of 400 mg X4P-001-IO once daily with 5 mg Inlyta twice daily has been selected for the Phase 2 portion of the ongoing Phase 1/2 study.

“We are encouraged by the results to date in this first cohort of patients, many of whom have been on study for over six months and have seen early signs of clinical efficacy with manageable side effects,” said Sudha Parasuraman, MD, Chief Medical Officer of X4. “We look forward to sharing a comprehensive update on the ongoing Phase 2a clinical trial, as well as the path forward for further development, in 2018.”

The Phase 2 portion of the study continues to enroll patients to evaluate the clinical efficacy of X4P-001-IO as measured by objective response rate (ORR), duration of response (DOR), and progression free survival (PFS), as well as explore the correlation of biomarkers with efficacy. (https://clinicaltrials.gov/ct2/show/NCT02667886)

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.

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.[1] 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.[2] 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.