Initial Clinical Trial Data for Combination of X4P-001-IO and Inlyta® (Axitinib) Demonstrate Encouraging Disease Control Rates and Durable Clinical Responses in Patients with Clear Cell Renal Cell Carcinoma (ccRCC)

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Combination of CXCR4 inhibition plus Inlyta® was generally well tolerated

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

CAMBRIDGE, Mass., Sept. 11, 2017 – X4 Pharmaceuticals, a clinical stage biotechnology company developing a novel CXCR4 inhibitor drug to improve immune cell trafficking to treat cancer and rare diseases, today announced Phase 1 results from the company's ongoing Phase 1/2 study of X4P-001-IO. The data were presented at the European Society of Medical Oncology (ESMO) 2017 Congress being held September 8-12 in Madrid, Spain.

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 were presented at the ESMO 2017 Congress on Sunday, September 10. All patients had received at least one prior line of therapy and 69 percent of patients have received at least two prior lines of therapy. Highlights of the poster presentation include:

- In the evaluable patient population, the combination of X4P-001-IO and Inlyta® produced a disease control rate (DCR) and objective response rate (ORR) of 92 percent (11/12) and 25 percent (3/12), respectively, including 3 partial responses (PRs).
- The median duration on treatment was 14.7 weeks and 43 percent of patients had been exposed to study treatment for at least 24 weeks.
- X4P-001-IO in combination with Inlyta® was generally well tolerated. The most frequent treatment-related adverse events (AEs) in patients receiving X4P-001-IO at 200 mg twice daily, 400 mg once daily, or 600 mg once daily were diarrhea, hypertension, fatigue, nausea, headache, decreased appetite, and vomiting. No grade 4 or 5 AEs occurred.
- 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.

"The high disease control rate and clinical responses in previously treated patients with late-stage clear cell renal cell carcinoma underscore the rationale for investigating the therapeutic potential of CXCR4 inhibition plus VEGFR inhibition," said Michael Atkins, MD, Deputy Director, Georgetown-Lombardi Comprehensive Cancer Center in Washington, DC and William M. Scholl Professor of, Oncology at Georgetown University School of Medicine. "The preliminary results shown in this clinical study are very encouraging and support continued investigation of this approach."

"By leveraging data from prior clinical studies, we were able to quickly reach the recommended Phase 2 dose of X4P-001-IO, and the combination has demonstrated good tolerability with early signs of clinical activity," said Sudha Parasuraman, MD, Chief Medical Officer of X4. "We look forward to presenting the full results of this study 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 exploring the correlation of biomarkers with efficacy.

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.

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

[2] Kidney Cancer Association, "Therapies for Advanced Kidney Cancer," http://www.kidneycancer.org/knowledge/learn/therapies-for-advanced-kidney-cancer/

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