



## Updated Clinical Data from Combination of X4P-001-IO and Inlyta® (axitinib) in Patients with Clear Cell Renal Cell Carcinoma Will Be Presented at the AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics

October 25, 2017

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**CAMBRIDGE, Mass., October 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 that the American Association for Cancer Research (AACR) 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. Updated preliminary efficacy data along with safety and tolerability of the combination will be highlighted in a poster presentation at the 2017 AACR-NCI-EORTC Molecular Targets and Cancer Therapeutics Conference on October 26-30 in Philadelphia, Pennsylvania.

### **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: Atkins, Michael

Session Category: Tumor Microenvironment

Session Date and Time: Sunday Oct 29, 2017 12:30 PM – 4:00 PM

Location: Hall E, Pennsylvania Convention Center

Permanent Abstract Number: B201

### **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.<sup>1</sup> 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.<sup>2</sup> 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.

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

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

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