X4 Pharmaceuticals Reports Positive Clinical Data from Phase 2 Expansion Study of X4P-001-IO and Axitinib in Patients with Clear Cell Renal Cell Carcinoma

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Favorable response rates and combinability demonstrated in heavily pretreated patients

Results presented at the 2018 American Society for Clinical Oncology (ASCO) Annual Meeting

CAMBRIDGE, Mass., June 2, 2018 – X4 Pharmaceuticals, a clinical stage biotechnology company developing novel CXCR4 antagonists to improve immune cell trafficking to treat cancer and rare diseases, today announced positive clinical results from the Phase 2 expansion 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 were the first from the Phase 2 portion of the study and demonstrated that the combination was well tolerated with a manageable safety profile and had encouraging response in heavily pretreated patients. In patients with ccRCC, the combination treatment of X4P-001-IO, a CXCR4 antagonist, and Inlyta, Pfizer's VEGFR kinase inhibitor, showed an objective response rate (ORR) of 23%, including 1 patient with a confirmed complete response (CR). Nearly 75% of patients received at least two prior lines of therapy prior to entering the study. The data were presented at the 2018 American Society of Clinical Oncology (ASCO) Annual Meeting on June 2nd in Chicago, IL.

"X4P represents a novel targeted mechanism of action with demonstrated tolerability and promising efficacy in combination with axitinib in patients with pretreated renal cancer. The results from this study demonstrate that X4P-001-IO has the potential to enhance clinical responses to axitinib and other tyrosine kinase inhibitors that target tumor angiogenesis," said Ulka Vaishampayan, MD, Chair, Karmanos Cancer Center, Professor of Oncology at Wayne State University, and lead investigator of the study.

Results from the 65 patients with advanced ccRCC enrolled in the ongoing study (as of the data cutoff date of March 23, 2018) were presented at ASCO and highlights of the poster presentation include:

- The combination of 400 mg X4P-001-IO administered once daily and 5 mg axitinib twice daily was well tolerated with a manageable safety profile. The most frequent treatment-related adverse events (AEs) were diarrhea, decreased appetite, fatigue, hypertension, nausea, headache and cough. No grade 4 or 5 AEs were observed.
- In the 47 evaluable patients, the overall response rate (ORR) was 23% with one patient achieving a confirmed complete response (CR). Response data from the remaining 18 patients is pending.
- Thirteen patients remain on study for 24 weeks or more; the median duration on treatment was 16 weeks (range 2 – 96 weeks).

"These interim results represent an important step in the continued development of X4P-001-IO. In this larger patient population, where many patients are still very early in treatment with the combination, we find promising signs of clinical efficacy. The tumor microenvironment modulating effect of X4P-001-IO is expected to increase and deepen responses over time, and we look forward to the maturation of the data in the coming months," said Sudha Parasuraman, MD, Chief Medical Officer of X4. "Our combined clinical experience continues to demonstrate the important role that CXCR4 antagonism may play in improving outcomes in combination with important cancer therapeutic modalities."

The Phase 2 portion of the study continues to follow patients on study 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). (https://clinicaltrials.gov/ct2/show/NCT02667886)

About X4P-001-IO in Cancer
X4P-001-IO is an investigational selective, oral, small molecule antagonist of C-X-C receptor type 4 (CXCR4). CXCR4 is a chemokine receptor present in abundance on certain immune cells and cancer cells and it plays a critical role in immune cell trafficking, infiltration and activation in the tumor microenvironment. 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. X4P-001-IO has the ability to help restore immunity within the tumor microenvironment and has the potential to enhance the anti-tumor activity of approved and emerging oncology agents, such as checkpoint inhibitors and targeted therapies. X4P-001-IO is being investigated in several clinical studies in solid tumors.

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 antagonize the CXCR4 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 clinical studies in solid tumors. 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. For more information, visit x4.theyatesnetwork.com.

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