X4 Pharmaceuticals Presents Clinical Data Showing Combination of X4P-001 and Opdivo® (nivolumab) Improved Clinical Responses in RCC Patients Unresponsive to Opdivo Monotherapy

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Findings with combination therapy of anti-PD-1 and CXCR4 antagonist in patients with clear cell renal cell carcinoma (ccRCC) presented in Poster Discussion session at ESMO 2018

Cambridge, MA – October 19, 2018 – X4 Pharmaceuticals, a clinical stage biotechnology company developing novel CXCR4 antagonists to improve immune cell trafficking to treat cancer and rare disease, today announced results from a pilot study of X4P-001-IO in combination with Opdivo® (nivolumab) in patients with clear cell renal cell carcinoma (ccRCC) who are non-responsive to the anti-PD-1 checkpoint inhibitor Opdivo alone. The data will be presented at a Poster Discussion session at the European Society for Medical Oncology (ESMO) 2018 Congress, taking place October 19-23, 2018, in Munich, Germany.

"The data from this study demonstrate that the combination with X4P-001-IO and nivolumab has the potential to augment responses in patients who previously received the anti-PD-1 checkpoint inhibitor nivolumab alone," said Toni K. Choueiri, M.D. Director, Lank Center for Genitourinary Oncology, Dana-Farber Cancer Institute, and lead investigator of the study. "This pilot study data requires validation in larger studies as we continue to seek treatments to address the larger population of cancer patients who do not adequately respond to checkpoint inhibitors."

Results were presented from the nine patients with advanced ccRCC enrolled in the pilot study (as of August 1, 2018) who were non-responsive to single agent Opdivo with either stable or progressive disease. Enrolled patients received X4P-001-IO (400 mg, oral, once daily) and continued to receive standard bi-weekly Opdivo therapy. Median duration of treatment with the combination was 3.7 months (range 1-15 months).

Highlights of the data presented at ESMO include:

- X4P-001-IO in combination with Opdivo was tolerable in ccRCC patients. The most frequent drug related adverse events were diarrhea, nasal congestion, ALT/AST increase, dry eye, fatigue. No grade 4 or 5 adverse events occurred. All Grade 3/serious adverse events were manageable with appropriate intervention.
- Combination therapy with X4P-001-IO and Opdivo exhibited anti-tumor activity in some patients with advanced ccRCC who were previously unresponsive to Opdivo monotherapy.
    - Four patients who had progressed on prior Opdivo monotherapy had a best response of stable disease with the additional X4P-001-IO to Opdivo treatment.
    - Of the five patients who were stable on prior Opdivo monotherapy, one had a partial response with combination therapy of X4P-001-IO and Opdivo.
    - Serum biomarker analyses identified significant early changes in cytokines and chemokines, including CXCL9, a chemoattractant ligand for cytotoxic T cell migration.

"These findings add to our clinical experience with X4P-001-IO and our growing understanding of combining CXCR4 antagonists with other agents, such as checkpoint inhibitors," said Ken Gorelick, M.D., Chief Medical Officer of X4 Pharmaceuticals. "X4 continues to explore the important role that CXCR4 antagonism may play in augmenting anti-tumor response in combination with other cancer therapeutic modalities, and therefore, potentially improve outcomes for cancer patients."

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 rare diseases and cancer. 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 is in a Phase 2/3 clinical trial in patients with WHIM syndrome, a rare genetic, primary immunodeficiency disease, and is currently under investigation in multiple clinical trials 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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