X4 Pharmaceuticals Presents Clinical Data Demonstrating Combinability of X4P-001-IO and Opdivo® (nivolumab) in Patients with Clear Cell Renal Cell Carcinoma

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Findings from a pilot study showed combination therapy of anti-PD-1 and CXCR4 antagonist had an acceptable safety profile with a potential to augment anti-tumor responses in patients who are non-responsive to anti-PD-1 checkpoint inhibitors alone

Cambridge, MA – May 15, 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 were presented at the 16th Annual Meeting of the Association for Cancer Immunotherapy (CIMT), taking place May 15-17 in Mainz, Germany.

Results from the nine patients with advanced ccRCC enrolled in the pilot study as of February 26, 2018 were presented at the CIMT meeting in a poster session on May 15th and an oral presentation on May 17th. All patients in the study were non-responsive to single agent Opdivo with either stable or progressive disease. Enrolled patients continued to receive standard bi-weekly Opdivo therapy and X4P-001-IO (400 mg, oral, once daily). Median duration of treatment with the combination was 3.7 months (range 1-10 months).

Highlights of the data presented at CIMT include:

- X4P-001-IO in combination with Opdivo had acceptable toxicity. The most frequent adverse events were diarrhea, nasal congestion, dry eye, headache and cough. 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 non-responsive to single agent Opdivo therapy.
    - Four patients who had progressed on prior Opdivo monotherapy had a best response of stable disease with additional X4P-001-IO treatment.
    - Of the five patients who were stable on prior Opdivo monotherapy, one had a partial response with combination therapy.

“These data 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 David F. McDermott, M.D., Beth Israel Deaconess Medical Center, Harvard Medical School and lead investigator of the study. “This preliminary 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.”

“These findings contribute to our growing understanding of combining CXCR4 antagonists with other agents such as checkpoint inhibitors,” said Sudha Parasuraman, M.D., Chief Medical Officer of X4 Pharmaceuticals. “Because the mechanisms of CXCR4 antagonism and check point inhibition act at different points in the tumor immunity cycle, it is reasonable to consider the potential for synergistic activity.”

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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