First Clinical Data from Combination of X4P-001-IO and Keytruda® (pembrolizumab) in Patients with Melanoma Will Be Presented at the Society for Immunotherapy of Cancer (SITC) Annual Meeting

November 3, 2017

Second presentation highlighting therapeutic benefit of CXCR4 inhibition in pre-clinical model of melanoma will also be presented

CAMBRIDGE, Mass., November 3, 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 first data from its ongoing Phase 1b clinical study of X4P-001-IO in patients with melanoma will be presented at the Society for Immunotherapy of Cancer (SITC) Annual Meeting on November 8-12 in National Harbor, MD. A second poster highlighting the effects of CXCR4 inhibition in a syngeneic model of melanoma will also be presented.

The details of the poster presentations are as follows:

X4P-001, an Orally Bioavailable CXCR4 Antagonist, Increases T-cell Infiltration in Human Metastatic Melanoma
Friday, November 10, Abstract # P367, Session Category: Immune Modulation, Cytokines, and Antibodies

Efficacy and Mechanism of Action of CXCR4 Inhibition in B16-OVA Melanoma Model
Friday, November 10, Abstract # P356, Session Category: Immune Modulation, Cytokines, and Antibodies

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. X4P-001-IO is being investigated in three separate clinical studies in solid tumors.

About Melanoma

Cutaneous malignant melanoma is the fifth most common cancer in men and the sixth most common cancer in women in the United States. When discovered early, melanoma is highly curable with 10-year overall survival rates approaching 95% for stage I melanoma and 45-77% for stage II melanoma.[1] However, for patients with stage III and IV melanoma, the prognosis is much worse. The 10-year survival rate for stage IV melanoma is 10-15%.[2] Adjuvant therapies for patients with resectable stage III melanoma include immunomodulating drugs, such as high dose interferon-α therapy and anti-CTLA-4 or PD-1 antibody therapy. Unmet medical needs remain to establish and improve overall survival in patients with advanced resectable melanoma, as well as improving objective response rates in patients who do not respond to existing treatments.

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, "SEER Stat Fact Sheets: Melanoma of the Skin," http://seer.cancer.gov/statfacts/html/melan.html

[2] National Cancer Institute, "What are the survival rates for melanoma skin cancer, by stage?," http://www.cancer.org/cancer/skincancer-melanoma/detailedguide/melanoma-skin-cancer-survival-rates

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