First Clinical Data for X4P-001-RD Demonstrating Preliminary Activity in Patients with WHIM Syndrome will be Presented at American Society for Hematology Annual Meeting

November 16, 2017

CAMBRIDGE, Mass., November 16, 2017 – X4 Pharmaceuticals, a clinical stage biotechnology company developing novel CXCR4 inhibitor drugs to improve immune cell trafficking to treat cancer and rare disease, today announced that Dr. David C. Dale, MD will present the first clinical data from an on-going study of X4P-001-RD in patients with WHIM Syndrome, a sub-type of a primary immunodeficiency disease, at the 59th Annual Meeting of the American Society for Hematology (ASH). Preliminary data from these abstracts are available on the ASH conference website. The details of the poster presentation is as follows:

X4P-001: A Novel Molecularly-Targeted Oral Therapy for WHIM Syndrome

Saturday, December 9, 2017, Abstract # 995, Presenter: David C. Dale, MD

"We are very pleased to share preliminary data showing that X4P-001-RD has a meaningful impact on the levels of circulating white blood cells in this severely immunodeficient patient population," said Paula Ragan, PhD, President and CEO of X4. "These data demonstrate the potential of X4P-001-RD to benefit patients with WHIM who otherwise have no approved treatment options and support our goal of advancing toward a pivotal study."

About WHIM Syndrome

WHIM syndrome is a primary immunodeficiency disease ("PID") caused by genetic mutations in the CXCR4 receptor gene resulting in susceptibility to certain types of infections. WHIM is an abbreviation for the characteristic clinical symptoms of the syndrome: Warts, Hypogammaglobulinemia, Infections, and Myelokathexis. Within the overall category of primary immunodeficiencies, there are between 15,000 and 100,000 patients in the US that are classified with PID of unknown origin — of which WHIM is one.[1,2,3] WHIM syndrome is a rare disorder and the precise prevalence or incidence of patients that have the genetic mutation responsible for WHIM syndrome is unknown. Because patients are highly susceptible to infections, WHIM syndrome is associated with significant morbidity beginning in early childhood and continuing throughout life. Current therapy is limited to treatment of acute infections with antibiotics or prevention through the use of intravenous immunoglobulin or G-CSF. There is no approved therapy for the treatment of WHIM syndrome.

About X4P-001-RD for Primary Genetic Immunodeficiency Disease

X4P-001-RD, an oral, small molecule inhibitor of CXCR4, or C-X-C receptor type 4, is being developed for use as a life-long treatment for patients with WHIM syndrome and other primary genetic immunodeficiencies. X4P-001-RD is currently being studied in a Phase 2/3 trial in patients with WHIM syndrome. Within the bone marrow, a normally functioning CXCR4 receptor controls the release of neutrophils and leukocytes into the blood stream, thereby ensuring normal immune surveillance functions throughout the body. In patients with WHIM syndrome, mutations to the CXCR4 receptor cause aberrant signaling leading to retention of neutrophils and leukocytes in the bone marrow and inadequate immune surveillance and function.[4,5] X4P-001-RD is designed to normalize the signaling for the mutant CXCR4 receptor to promote the release of neutrophils and leukocytes, thereby restoring healthy immunity.

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] Boyle JM, Buckley, RH, Population Prevalence of Diagnosed Primary Immunodeficiency Diseases in the United States. *J Clin Immunol* 2007;27:497–502.

[2] Gathmann B, Grimbacher B, et al. The European internet-based patient and research database for primary immunodeficiencies: results 2006–2008. *Clin Exp Immunol*. 2009 Sep;157 Suppl 1:3-11.

[3] Modell V, Gee B, et al. Global study of primary immunodeficiency diseases (PI) — diagnosis, treatment, and economic impact: an updated report from the Jeffrey Modell Foundation. *Immunol Res*. 2011;51:61–70.

[4] Hernandez PA, Gorlin RJ, Lukens JN, et al. Mutations in the chemokine receptor gene CXCR4 are associated with WHIM syndrome, a combined immunodeficiency disease. *Nature Genetics* 2003;34(1):70-74.

[5] Gulino AV, Moratto D, Sozzani S, et al. Altered leukocyte response to CXCL12 in patients with Warts Hypogammaglobulinemia, Infections, Myelokathexis (WHIM) syndrome. *Blood* 2004;104(2):444-452.