X4 Pharmaceuticals to Present Clinical Data from Phase 2 Study of X4P-001-RD in WHIM syndrome

May 17, 2018

Study designed to evaluate X4P-001-RD in a rare genetic, primary immunodeficiency disease to be presented at the Annual Congress of the European Hematology Association

Cambridge, MA – May 17, 2018 – 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 an abstract highlighting X4P-001-RD, the company's CXCR4 antagonist, has been selected for poster presentation at the 23rd Annual Congress of the European Hematology Association (EHA), taking place June 14-17 in Stockholm, Sweden. The presentation will describe interim clinical results from the ongoing Phase 2/3 study of X4P-001-RD in patients with WHIM syndrome, a rare genetic, primary immunodeficiency disease.

Details of the presentation on X4P-001-RD in WHIM syndrome are as follows:

Title: Phase 2 Study of X4P-001: A Targeted Oral Therapy for Patients with WHIM Syndrome

Author: David Dale, M.D., University of Washington

Abstract #: PS1056

Poster Session: Bone marrow failure syndromes incl. PNH – Clinical

Date and Time: Saturday, June 16, 5:30-7:00 p.m. CEST

About WHIM Syndrome
WHIM syndrome is a primary immunodeficiency disease caused by genetic mutations in the CXCR4 receptor generesulting 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 primary immunodeficiency disease 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 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.

[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.

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