X4 Pharmaceuticals Presents Additional Positive Phase 2 Results for X4P 001 in WHIM Syndrome, Continues on Path to Initiation of Phase 3 Trial

December 1, 2018

X4 is advancing clinical and commercial plans for X4P-001 in a rare genetic, primary immunodeficiency disease that currently has no approved treatments

Additional Phase 2 results presented at the 60th Annual Meeting of the American Society of Hematology

CAMBRIDGE, MA – December 1, 2018 – X4 Pharmaceuticals, a clinical stage biotechnology company developing novel CXCR4 allosteric antagonist drugs to improve immune cell trafficking to treat rare diseases and cancer, today announced the presentation of additional Phase 2 clinical data demonstrating a positive safety profile and clinical activity of X4P-001 in patients with WHIM syndrome, a rare primary immunodeficiency disease. The clinical results are being presented today in a poster session at the 60th American Society of Hematology (ASH) Annual Meeting taking place in San Diego.

"We continue to see encouraging results from this Phase 2 trial, as X4P-001 drug exposure appears to correlate with increases in absolute neutrophil count and absolute lymphocyte count levels. Increases in these biomarkers are an indication of potential improvement in the pathophysiology underlying WHIM syndrome. An example of clinical improvement and impact on symptoms was the continued reduction in a WHIM patient's human papillomavirus-related warts following X4P-001 therapy," said David C. Dale, MD, Professor of Medicine at the University of Washington School of Medicine, Seattle, WA, and lead investigator of the trial.

The poster presentation at ASH describes results from the first 8 patients with genetically confirmed WHIM syndrome and demonstrates the dose-dependent biomarker activity used to support dose selection for the Phase 3 trial. Additional updates on safety and clinical activity across all patients who were enrolled as of the August 17, 2018 data-cut off are also presented in the poster.

Information about the clinical trial of X4P-001 in patients with WHIM syndrome can be found on clinicaltrials.gov: https://clinicaltrials.gov/ct2/show/NCT03005327.

"X4P-001 in WHIM syndrome is X4's lead product candidate and our team is rapidly advancing toward the Phase 3 trial, with the goal of delivering X4P-001 to the global community of WHIM patients who currently lack any approved treatment specifically for this disease," said Paula Ragan, PhD, president and chief executive officer of X4 Pharmaceuticals. "We are confident in our path forward based on these favorable Phase 2 results, and we look forward to starting the pivotal Phase 3 trial in the first half of 2019."

About WHIM Syndrome
WHIM syndrome is a primary immunodeficiency disease 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 U.S. who 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. Individuals with WHIM syndrome are more susceptible to potentially life-threatening bacterial infections.[4] Additionally, 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 of infections 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 rare diseases and cancer. X4'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, is in a Phase 2 clinical trial in patients with WHIM syndrome, a rare genetic, primary immunodeficiency disease, and is currently also under investigation in multiple clinical trials in oncology. X4 expects to begin a Phase 3 trial of X4P-001 in WHIM syndrome in the first half of 2019. 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 www.x4pharma.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.

[4] Primary Immunodeficiency Foundation: https://primaryimmune.org/disease/whim-syndrome

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