X4 Pharmaceuticals Receives Orphan Drug Designation from the U.S. FDA for X4P-001-RD for the Treatment of WHIM Syndrome

October 15, 2018

CAMBRIDGE, Mass. October 15, 2018 – X4 Pharmaceuticals, a clinical stage biotechnology company developing novel *CXCR4* antagonist drugs, today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation for the Company's drug candidate, X4P-001-RD for the treatment of WHIM syndrome, a primary immunodeficiency disease for which there are currently no approved treatments for patients. X4P-001-RD is currently in a Phase 2/3 clinical trial in patients with WHIM syndrome, and positive interim results from the Phase 2 portion of the study were announced at the European Hematology Association Congress in June 2018.

"There are currently no approved therapies to address the underlying cause and significant needs of patients with WHIM. This Orphan Drug Designation by FDA is an important milestone in our clinical development of X4P-001-RD as a new treatment option for patients with WHIM syndrome," said Paula Ragan, PhD, Chief Executive Officer of X4. "We are excited to continue to work with leaders, experts and patient advocates addressing primary immunodeficiency disease to raise awareness for WHIM diagnosis and treatment and to bring X4P-001-RD to patients suffering with this disease."

Orphan Drug Designation is granted by the FDA to drugs and biologics that are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases or conditions that affect fewer than 200,000 people in the United States. Orphan Drug Designation provides certain incentives which may include tax credits towards the cost of clinical trials and prescription drug user fee waivers. If a product that has Orphan Drug Designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan product exclusivity.

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 U.S. 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. 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 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. 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 is in a Phase 2/3 clinical trial in patients with WHIM syndrome, a rare genetic, primary immunodeficiency disease, and is currently under investigation in multiple clinical trials 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.
4. Primary Immunodeficiency Foundation: https://primaryimmune.org/disease/whim-syndrome

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