X4 Pharmaceuticals Secures $27 Million in Series B Financing

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Proceeds Will Support Advancement of a Pivotal Study in a Rare Disease and Multiple Proof of Concept Studies in Immuno-Oncology

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 diseases, today announced the successful completion of a $27 million Series B financing. Proceeds from the financing will be used to advance X4's two lead drug candidates in clinical efficacy studies in immuno-oncology and a pivotal study in WHIM syndrome, a rare primary immunodeficiency disease. X4's novel therapeutics are directed against the CXCR4 pathway to correct the trafficking of key immune cells that regulate healthy immune surveillance throughout the body.

"We are incredibly pleased to have the support of Cormorant Asset Management and additional new and existing investors to support our mission of bringing innovative treatments to patients with cancer and rare diseases," said Paula Ragan, PhD, President and CEO of X4. "We are now in a strong position to advance our lead candidate in WHIM syndrome into a pivotal study in 2018 and to advance our proof of concept studies in oncology. This financing recognizes the progress we have made as well as the important milestones that lie ahead in 2018."

"Having been an investor in the earliest stages of X4, we have witnessed the strong progress the company has made and the impact these treatments are having for patients," said Bihua Chen, CEO and Portfolio Manager, Cormorant Asset Management. "X4 has built a differentiated approach rooted in strong science and clear regulatory pathways. We are pleased to support X4's continuing work in developing and commercializing innovative therapies for primary immunodeficiencies and cancer."

In the two years since X4 launched in 2015, the company has rapidly entered clinical studies with lead programs for two different diseases: as an immuno-oncology agent for solid tumors and as a treatment for the rare disease WHIM syndrome. The company's oral, small molecule drug candidates inhibit the binding of chemokine CXCL12 to C-X-C receptor type 4 (CXCR4), a receptor-ligand pair that plays an essential role in normal immune surveillance. X4P-001-IO has demonstrated encouraging results in Phase 1/2 testing in refractory clear cell renal cell carcinoma, as well as in melanoma where results support activation of the immune system. In addition, X4P-001-RD has demonstrated initial proof-of-concept in the Phase 2 portion of an on-going Phase 2/3 study in patients with WHIM syndrome.

About Renal Cell Carcinoma

Kidney cancer is among the ten most common cancers in both men and women with more than 60,000 new diagnoses each year in the United States.[1] Clear cell renal cell carcinoma (ccRCC) is the most common form of kidney cancer, and advanced ccRCC accounts for approximately 20% of the patient population. Therapies for advanced ccRCC include immunotherapies, mammalian target of rapamycin (mTOR) kinase inhibitors, and angiogenesis inhibitors, such as vascular endothelial growth factor (VEGF) inhibitors.[2] There continue to be unmet medical needs with advanced ccRCC because durable responses remain a serious clinical challenge for patients with advanced disease.

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.[3,4,5] 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 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, "Surveillance, Epidemiology, and End Results Program," http://seer.cancer.gov/statfacts/html/kidrp.html

[2] Kidney Cancer Association, "Therapies for Advanced Kidney Cancer," http://www.kidneycancer.org/knowledge/learn/therapies-for-advanced-kidney-cancer/

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

[4] 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.

[5] 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.