X4 Pharmaceuticals Unveils Development Strategy for CXCR4 Inhibitor Pipeline in Cancer Supported by $37.5 million Series A Financing

Company to advance multiple CXCR4 programs focused on cancer immunotherapy, including advancing lead candidate X4P-001 into two clinical studies in refractory cancers

December 14, 2015 07:00 AM Eastern Standard Time

CAMBRIDGE, Mass.--(BUSINESS WIRE)--X4 Pharmaceuticals today announced the launch of the company's development strategy for its pipeline of C-X-C receptor type 4 (CXCR4 inhibitors), including two clinical studies initiating in 2016 in refractory cancers with its lead drug candidate, X4P-001. The formation of X4 is based on drug compounds that originate from a portfolio of oral CXCR4 inhibitors exclusively licensed from Sanofi.

Development of X4's CXCR4 inhibitors will be funded initially through a $37.5 million Series A financing, which the Company recently closed. Maxim Merchant Capital, a wholly owned division of Maxim Group LLC, served as the sole placement agent for the financing, with Cormorant Asset Management serving as lead investor.

Henri Termeer, the former Chairman, president and CEO of Genzyme, and a founding advisor and investor in X4, stated, "X4 has assembled the expertise, drug assets and strategies to build extraordinary value for its constituents, including the potential to have a significant impact on the treatment of cancer."

X4 will launch multiple clinical studies with X4P-001 in 2016 in advanced cancers, including refractory clear cell renal cell carcinoma (ccRCC) and a second solid tumor indication. A second drug program, X4P-002, which is currently in pre-clinical development, is being optimized for the treatment of brain cancers, and is expected to enter the clinic in 2017. Inhibition of CXCR4, a receptor over-expressed in many cancers, is designed to block non-cancerous immuno-suppressive and pro-angiogenic cells from populating the tumor, thereby disrupting the cancer microenvironment and restoring normal immune surveillance functions. The novel mechanism of CXCR4 inhibition increases the ability of T-Cells to track and destroy cancer.

"CXCR4 inhibition is a novel approach to immunotherapy whose potential we are only beginning to explore for the treatment of cancer," said Keith Flaherty, MD, the director of the Henri and Belinda Termeer Center for Targeted Therapies at the Massachusetts General Hospital and co-founder of X4. "Immune system

surveillance is the front line of keeping cancer in check and CXCR4 plays a key role in the trafficking of immunosuppressive cells. Inhibition of this receptor may be beneficial in addressing a broad range of cancers, offering the potential to improve both the magnitude and durability of treatment responses."

The development of X4's clinical candidates will be led by the company's seasoned management team and founders, who bring unique experience in the study the CXCR4 pathway. The team is led by Chief Executive Officer Paula Ragan, PhD, who brings more than 15 years of experience in senior leadership roles at biotechnology companies, including Genzyme. Other members of the team include:

- Robert Arbeit, MD, Senior Vice President of Clinical Development and Translational Research, who served in leadership roles at Idera Pharmaceuticals, Paratek Pharmaceuticals and Cubist Pharmaceuticals;
- Alison Lawton, consulting Chief Operating Officer, who has 30 years of global biopharmaceutical experience, including 21 years at Genzyme where she led the regulatory team for the approval of plerixafor, a CXCR4 inhibitor, in 2008.
- Alan Walts, PhD, co-founder of X4 and Interim Chairman, spent over 25 years with Genzyme, where his roles included President of Genzyme Pharmaceuticals, Senior Vice President of Corporate Development, and Managing Director of Genzyme Ventures

"With a strong financial foundation and leadership team in place, X4 is well positioned to bring forward CXCR4 inhibitors as an innovative new immunotherapy treatment approach for a broad spectrum of cancers," said Dr. Ragan. "We look forward to executing on our plans for advancing our CXCR4 pipeline programs, and to seeing the early promise of this approach translate in the clinic."

X4's scientific founder is Renato Skerlj, PhD, whose work in immune-mediated drug discovery led him to become an inventor of both plerixafor, a stem cell mobilizer approved in 2008, and ertapenem, an anti-bacterial approved in 2001. Dr. Skerlj has over 20 years of pharmaceutical experience in the discovery and development of novel therapies. Most recently, he was the Head of Small Molecule Discovery at Genzyme and prior to joining Genzyme, was part of the executive team at AnorMED, a publicly-traded company acquired by Genzyme for $580 million in 2006.

About X4 Pharmaceuticals

X4 Pharmaceuticals is developing novel therapeutics designed to improve immune cell trafficking and increase the ability for T-Cells to track and destroy cancer cells. The company's oral small molecule drug candidates inhibit the CXCR4 receptor, a pathway which plays a central role in promoting the immunosuppressive and pro-angiogenic microenvironment of many cancers. X4P-001, the company's lead program, is expected to enter Phase 1/2 testing in refractory clear cell renal cell carcinoma (ccRCC) and other solid tumor indications, and its second program, X4P-002, is in pre-clinical development for oncology applications. 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.

Contacts

The Yates Network
Kathryn Morris, 845-635-9828
kathryn@theyatesnetwork.com