Precigen Announces First Patient Dosed in Phase 1 Clinical Study of INXN-4001

May 21, 2018

Milestone represents the first ever triple effector gene therapy for heart failure

GERMANTOWN, Md., May 21, 2018 /PRNewswire/ -- Precigen, Inc., a wholly-owned subsidiary of Intrexon Corporation and a biopharmaceutical company specializing in the development of innovative gene and cellular therapies to improve the lives of patients, today announced the treatment of the first patient in a Phase 1 first-in-human clinical trial of its investigational therapy, INXN-4001.

"This start of dosing is an exciting and important milestone for Precigen as INXN-4001 is the first investigational new drug (IND) application filed and the first patient dosed for Precigen as a newly formed wholly-owned subsidiary of Intrexon," said Helen Sabzevari, PhD, president of Precigen. "This milestone represents an additional first for Precigen and for heart failure patients as it is also the first use of the retrograde coronary sinus infusion (RCSI) procedure in left ventricular assist device (LVAD) patients."

INXN-4001 is a non-viral, multigene plasmid designed to express three different human proteins. A unique triple-effector plasmid, INXN-4001 addresses multiple malfunctions of cardiomyocytes – which are the muscle cells that make up the heart tissue -- in patients with heart failure and is being studied clinically to assess its safety and feasibility of minimally invasive, RCSI in LVAD patients.

This first INXN-4001 clinical study (clinical trial number: NCT03409627) is designed to evaluate the safety of INXN-4001 in LVAD patients as assessed by the incidence rate of all adverse events occurring up to six months post-treatment. The study will also examine the ability of LVAD patients to wean from the LVAD device, their quality of life and their overall daily activity.

"We are very pleased to begin this ground-breaking study of the use of three simultaneous effectors, each of which has shown benefit in the treatment of heart failure. It promises to open a new approach to combination therapy which we hope to show will enhance the benefit provided to heart failure patients," said Les Miller, MD, FAHA, FACC, director of heart failure and gene therapy cardiovascular research at Baycare Health System.

Heart failure is a leading cause of death worldwide and projected to rise by 46 percent by 2030 according to the American Heart Association's 2017 Heart Disease and Stroke Statistics Update recent publication.

INXN-4001 represents the leading program in Precigen's majority-owned subsidiary Xogenex, LLC, a company focused on the development of multi-genic biological therapies for cardiovascular disease.

Precigen : Advancing Medicine with Precision™
Founded in 2017, Precigen is a dedicated discovery and clinical stage biopharmaceutical company advancing the next generation of gene and cellular therapies using precision technology to target the most urgent and intractable diseases in oncology, autoimmune disorders, and emerging specialty therapy areas. Our technologies enable us to find innovative solutions for affordable biotherapeutics in a controlled manner. Precigen operates as an innovation engine progressing a preclinical and clinical pipeline of well-differentiated unique therapies toward clinical proof-of-confidence and commercialization. Precigen was founded as a wholly-owned subsidiary of Intrexon Corporation (NYSE: XON) and leverages Intrexon's proprietary technology platforms to advance human health. Learn more about Precigen at www.precigentherapeutics.com.

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