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Precigen Triple-Gene Provides Six-month Follow-up Data from Phase I Study of INXN-4001, a Multigenic Investigational Therapeutic Candidate for Heart Failure

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- **INXN-4001 delivered via retrograde coronary sinus infusion was well-tolerated in patients with chronic heart failure -**
- **Preliminary data suggest an overall improvement in patient reported outcomes in 50% of patients six months after treatment -**

GERMANTOWN, Md., Aug. 6, 2020 /PRNewswire/ -- Precigen Triple-Gene, a clinical stage cardiovascular gene therapy company and majority-owned subsidiary of [Precigen, Inc.](#) (Nasdaq: PGEN), today announced six-month follow-up data from the Phase I trial of INXN-4001 (clinical trial identifier: [NCT03409627](#)), a multigenic investigational therapeutic candidate under evaluation for the treatment of heart failure. The INXN-4001 investigational therapy uses a non-viral plasmid designed to constitutively express human SDF-1 α , VEGF165, and S100A1 gene products, which affect progenitor cell recruitment, angiogenesis, and calcium handling, respectively. INXN-4001 is designed to target the underlying molecular mechanisms of pathological myocardial remodeling and is delivered to the ventricle via retrograde coronary sinus infusion (RCSI).

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The primary objectives of the Phase I study are to evaluate the safety of INXN-4001 infusion into the myocardium of left ventricular assist device (LVAD) patients and to demonstrate safety and feasibility of RCSI. Secondary endpoints include improvements in 6-min walk test (6MWT) duration and distance and quality of life, as assessed by Kansas City Cardiomyopathy Questionnaire (KCCQ) responses.

Twelve chronic heart failure patients were treated with INXN-4001 in either Cohort 1 (80 mg/40 ml; n=6) or Cohort 2 (80 mg/80 mL; n=6) and 10 were evaluated for six-month follow-up. The study has met the primary endpoints to evaluate safety and feasibility for INXN-4001. The infusions of INXN-4001 were overall well tolerated, with no adverse events attributed to INXN-4001, whereas one serious adverse event was considered related to the infusion procedure.

Furthermore, preliminary assessment of the secondary endpoints show improvement by at least five points in total KCCQ score, a validated tool qualified by the FDA for Clinical Outcome Assessment, was observed in 50% of patients evaluable (5 out of 10) at the six-month follow-up visit. These included four patients treated in Cohort 1 and one patient treated in Cohort 2. Of the evaluable patients, 50% (4 out of 8) had an improvement in the 6MWT (> 50 meters in distance walked) at six months post-infusion compared to pre-treatment baseline.

"Overall the data support that INXN-4001 is well tolerated in patients with chronic heart failure on an LVAD," said Douglas L. Mann, MD, a leading cardiologist and key opinion leader in the field of heart failure. "Moreover, there was evidence of improvement in patient reported outcomes six months after treatment."

"Heart failure is a devastating disease with high unmet medical need. We are encouraged that INXN-4001 successfully met the primary safety and feasibility endpoints and that the preliminary assessment of the secondary endpoints show promising improvements in the quality of life six months after treatment for these patients," said Helen Sabzevari, PhD, President and CEO of Precigen. "We look forward to sharing the final study results at the 12-month interval."

About Precigen Triple-Gene

Precigen Triple-Gene, a majority-owned subsidiary of Precigen, Inc. (Nasdaq: PGEN), is a clinical stage gene therapy company focused on advancing targeted, controllable, and multigenic gene therapies for the treatment of complex cardiovascular diseases. The Company's lead product is a non-viral investigational gene therapy candidate that drives expression of three candidate effector genes involved in heart failure. Learn more about Precigen Triple-Gene at www.precigen.com/triple-gene/.

Precigen: Advancing Medicine with Precision™

Precigen (Nasdaq: PGEN) is a dedicated discovery and clinical stage biopharmaceutical company advancing the next generation of gene and cell therapies using precision technology to target the most urgent and intractable diseases in our core therapeutic areas of immuno-oncology, autoimmune disorders, and infectious diseases. 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-concept and commercialization.

For more information about Precigen, visit www.precigen.com or follow us on Twitter [@Precigen](#) and [LinkedIn](#).

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Some of the statements made in this press release are forward-looking statements. These forward-looking statements are based upon the Company's current expectations and projections about future events and generally relate to plans, objectives, and expectations for the development of the Company's business, including the timing and progress of preclinical and clinical trials and discovery programs, the promise of the Company's portfolio of therapies, the Company's refocus to a healthcare-oriented business, and its continuing evaluation of options for the Company's non-healthcare businesses. Although management believes that the plans and objectives reflected in or suggested by these forward-looking statements are reasonable, all forward-looking statements involve risks and uncertainties, including the possibility that the timeline for the Company's clinical trial might be impacted by the COVID-19 pandemic, and actual future results may be materially different from the plans, objectives and expectations expressed in this press release. The Company has no obligation to provide any updates to these forward-looking statements even if its expectations change. All forward-looking statements are expressly qualified in their entirety by this cautionary statement. For further information on potential risks and uncertainties, and other important factors, any of which could cause the Company's actual results to differ from those contained in the forward-looking statements, see the section entitled "Risk Factors" in the Company's most recent Annual Report on Form 10-K and subsequent reports filed with the Securities and Exchange Commission.

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