Precigen Receives Fast Track Designation for PRGN-3006 UltraCAR-T® in Patients with Relapsed or Refractory Acute Myeloid Leukemia

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– Acute myeloid leukemia (AML) is among the most common types of leukemia in adults –

– PRGN-3006 UltraCAR-T previously received orphan drug designation (ODD) in patients with AML by the US Food and Drug Administration (FDA) –

– PRGN-3006 UltraCAR-T has demonstrated a favorable safety profile to date with no dose-limiting toxicities or neurotoxicity, and has demonstrated dose-dependent in vivo expansion and durable persistence –

GERMANTOWN, Md., April 4, 2022 /PRNewswire/ -- Precigen, Inc. (Nasdaq: PGEN), a biopharmaceutical company specializing in the development of innovative gene and cell therapies to improve the lives of patients, today announced that the FDA has granted Fast Track designation for PRGN-3006 UltraCAR-T® in patients with relapsed or refractory (r/r) AML (clinical trial identifier: NCT03927261). PRGN-3006 was previously granted FDA Orphan Drug Designation.

PRGN-3006 UltraCAR-T is a multigenic autologous chimeric antigen receptor (CAR)-T cell treatment utilizing Precigen's non-viral *Sleeping Beauty* system to simultaneously express a CAR specifically targeting CD33, which is over expressed on AML blasts; membrane bound IL-15 for enhanced *in vivo* expansion and persistence; and a kill switch to conditionally eliminate CAR-T cells for an improved safety profile.

Precigen's UltraCAR-T platform is designed to overcome limitations of currently available CAR-T therapies by utilizing an advanced overnight non-viral gene delivery manufacturing process at a medical center's cGMP facility without the need for *ex vivo* expansion. Current CAR-T cell therapies are limited due to, *inter alia*, the prolonged interval between apheresis to product infusion and an exhausted phenotype of T cells resulting from lengthy *ex vivo* expansion. UltraCAR-T cells for the PRGN-3006 study are manufactured overnight using Precigen's proprietary UltraPorator™ system.

"We are very pleased to receive the FDA's Fast Track designation, which facilitates development and expedites the review process of drugs that address serious conditions and high unmet medical needs," said Helen Sabzevari, PhD, President and CEO of Precigen. "AML is a rapidly progressing disease with a very poor prognosis. The Fast Track designation will help facilitate the timely development of this program and we look forward to working more closely with the FDA to potentially bring this new and highly differentiated overnight UltraCAR-T therapy to patients."

About AML

AML is a cancer that starts in the bone marrow, but most often moves into the blood.[1] Though considered rare, AML is among the most common types of leukemia in adults.[2] In 2019, it was estimated that 21,450 new cases of AML would be diagnosed in the US.[2] AML is uncommon before the age of 45 and the average age of diagnosis is about 68.[2] The prognosis for patients with AML is poor with an average 5-year survival rate of approximately 25 percent overall, and less than a 5 percent 5-year survival rate for patients older than 65.[3] Amongst elderly AML patients (≥ 65 years of age), median survival is short, ranging from 3.5 months for patients 65 to 74 years of age to 1.4 months for patients ≥ 85 years of age.[3]

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 therapies toward clinical proof-of-concept and commercialization. For more information about Precigen, visit www.precigen.com or follow us on Twitter @Precigen, LinkedIn or YouTube.

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Cautionary Statement Regarding Forward-Looking Statements
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 studies, clinical trials, discovery programs and related milestones, the promise of the Company's portfolio of therapies, and in particular its CAR-T and AdenoVerse therapies. 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 trials 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.

References
[1] American Cancer Society. What is Acute Myeloid Leukemia (AML)?
[2] American Cancer Society. Key Statistics for Acute Myeloid Leukemia (AML)
[3] Thein, M., et al., Outcome of older patients with acute myeloid leukemia: an analysis of SEER data over 3 decades. Cancer, 2013. 119(15): p.2720-7

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