



## **Precigen Receives FDA Orphan Drug Designation for PRGN-3006 UltraCAR-T™ in Patients with Acute Myeloid Leukemia (AML)**

January 6, 2020

### **- Patients with AML have limited treatment options for this debilitating and lethal disease -**

GERMANTOWN, Md., Jan. 6, 2020 /PRNewswire/ -- Precigen, Inc., a biopharmaceutical company specializing in the development of innovative gene and cellular therapies to improve the lives of patients, today announced that the US Food and Drug Administration (FDA) has granted orphan drug designation (ODD) to PRGN-3006, a first-in-class investigational therapy using Precigen's non-viral UltraCAR-T™ therapeutic platform for patients with relapsed or refractory acute myeloid leukemia (AML) (clinical trial identifier: NCT03927261). Precigen announced in Q3 2019 that it had completed enrollment for the first cohort of this clinical trial and expects an initial data readout in the second half of 2020.

The FDA grants ODD status to medicines intended for the treatment, diagnosis or prevention of rare diseases or disorders that affect fewer than 200,000 people in the US. Medicines that receive the ODD designation may qualify for a number of incentives that help to expedite and reduce the cost of development, approval and commercialization.

"This regulatory designation underscores the critical medical need for new therapies to treat AML patients. AML is a progressive, debilitating and often fatal disease with limited treatment options," said Helen Sabzevari, PhD, President and CEO of Precigen\*. "As the first regulatory designation for our proprietary UltraCAR-T platform, this orphan drug designation helps to advance the PRGN-3006 investigational therapy and provides important incentives and support to deliver this medicine as rapidly as possible for those patients suffering from this disease."

PRGN-3006 utilizes Precigen's transformative UltraCAR-T therapeutic platform, which eliminates *ex vivo* expansion, reduces manufacturing time and provides the ability to administer CAR-T therapy to patients only one day after non-viral gene transfer at the cancer center. PRGN-3006 UltraCAR-T is a multigenic CAR-T cell treatment utilizing Precigen's advanced non-viral gene delivery system to co-express a chimeric antigen receptor, membrane-bound interleukin-15 (mblIL15), and a kill switch for better precision and control in targeting relapsed or refractory AML and higher risk MDS.

Preclinical data for PRGN-3006 UltraCAR-T™ evaluating non-viral, multigenic autologous CAR-T cells administered one day after gene transfer for the treatment of AML and MDS were presented at the 2019 American Society for Hematology (ASH) annual meeting and exposition in Orlando, Florida.

### **About Acute Myeloid Leukemia (AML)**

AML is a cancer that starts in the bone marrow, but most often moves into the blood<sup>1</sup>. Though considered rare, AML is among the most common types of leukemia in adults<sup>2</sup>. In 2019, it was estimated that 21,450 new cases of AML would be diagnosed in the US<sup>2</sup>. AML is uncommon before the age of 45 and the average age of diagnosis is about 68<sup>2</sup>. 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<sup>3</sup>. 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<sup>3</sup>.

### **About the FDA Orphan Drug Designation**

FDA orphan drug designation is granted to drugs intended to treat rare diseases or disorders that affect fewer than 200,000 people in the US. Medicines that receive the ODD designation may qualify for a number of incentives that help to expedite and reduce the cost of development, approval and commercialization, such as grants, clinical design support, FDA fee waivers, tax incentives, and seven years of market exclusivity.

### **Precigen : Advancing Medicine with Precision™**

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 immuno-oncology, autoimmune disorders, and infectious diseases. Precigen also follows the science opportunistically in pursuit of promising programs in emerging therapeutics. 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. Precigen leverages a diverse portfolio of technology platforms to advance human health. For more information about Precigen, visit [www.precigen.com](http://www.precigen.com) or follow us on Twitter @Precigen and LinkedIn.

\*Precigen's parent company, Intrexon Corporation (Nasdaq: XON), announced on January 2, 2020 that it will refocus the company on healthcare, will change its name to Precigen, Inc., and has appointed Helen Sabzevari, PhD, as President and CEO. The parent company will continue to hold, among its several health assets, all of Precigen's discovery and clinical stage technology and programs.

### **Precigen's UltraCAR-T™ Therapeutic Platform**

Precigen's UltraCAR-T platform has the potential to disrupt the CAR-T treatment landscape by increasing patient access through shortening manufacturing time, decreasing manufacturing-related costs, and improving outcomes using advanced approaches for precise tumor targeting and control of the immune system. The platform brings several key advancements: 1) Non-viral gene transfer using multigenic vectors for expression of multiple effector genes leads to better precision and control of tumor targeting and eliminates the need for virus; 2) Sustained persistence and desired phenotype of infused UltraCAR-T helps address T-cell exhaustion, a common issue with current CAR-T therapies; 3) T-cell control by incorporation of

kill switch technology to potentially improve the safety profile; and 4) Rapid manufacturing of UltraCAR-T cells using our proprietary non-viral gene transfer process, which eliminates the need for *ex vivo* propagation, thus dramatically reducing wait times for patients from weeks to fewer than two days.

**Safe Harbor Statement**

Some of the statements made in this press release are forward-looking statements. These forward-looking statements are based upon our current expectations and projections about future events and generally relate to plans, objectives and expectations for the development of our business, including the timing and progress of preclinical and clinical trials and discovery programs, and the anticipated refocusing and renaming of Precigen's parent company. 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 and actual future results may be materially different from the plans, objectives and expectations expressed in this press release.

<sup>1</sup> American Cancer Society. What is Acute Myeloid Leukemia (AML)? (Accessed January 2020)

<sup>2</sup> American Cancer Society. Key Statistics for Acute Myeloid Leukemia (AML) (Accessed January 2020)

<sup>3</sup> 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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