Ocugen Announces Rare Pediatric Disease Designation Granted for OCU410ST—Modifier Gene Therapy for the Treatment of Stargardt Disease

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MALVERN, Pa., May 27, 2025 (GLOBE NEWSWIRE) -- Ocugen, Inc. (Ocugen or the Company) (NASDAQ: OCGN), a pioneering biotechnology leader in gene therapies for blindness diseases, today announced that the United States Food and Drug Administration (U.S. FDA) has granted Rare Pediatric Disease Designation (RPDD) for OCU410ST for the treatment of *ABCA4*-associated retinopathies including Stargardt disease, retinitis pigmentosa 19, and cone-rod dystrophy 3. Previously, OCU410ST received Orphan Drug designations for the treatment of *ABCA4*-associated retinopathies from the FDA and European Medicines Agency.

"This latest designation for OCU410ST reaffirms the urgency of providing a therapeutic option to Stargardt patients who have no FDA-approved treatment available," said Dr. Shankar Musunuri, Chairman, CEO, and Co-founder of Ocugen. "This inherited retinal disease presents itself most often in childhood—making Stargardt disease a diagnosis that not only affects the patient but impacts the entire family."

The FDA grants RPDD for serious and life-threatening diseases that primarily affect children ages 18 years or younger and fewer than 200,000 people in the U.S. There are approximately 100,000 people in the U.S. and Europe combined living with Stargardt disease.

With this designation for OCU410ST, Ocugen may be awarded a Priority Review Voucher (PRV), if the PRV program is reauthorized by the U.S. Congress. The PRV program is designed to incentivize drug development for serious rare pediatric diseases. If awarded, a PRV can be redeemed to receive priority review for a different product or sold to another sponsor and typically sells for about $100 million.

Ocugen is committed to advancing the OCU410ST program through clinical development and plans to initiate the Phase 2/3 pivotal confirmatory trial in the next few weeks with a target Biologics License Application (BLA) filing in 2027.

About OCU410ST
OCU410ST utilizes an AAV delivery platform for the retinal delivery of the *RORA* (RAR-Related Orphan Receptor A) gene. It represents Ocugen's modifier gene therapy approach, which is based on Nuclear Hormone Receptor (NHR) *RORA* that regulates pathophysiological pathways linked to Stargardt disease, such as lipofuscin formation, oxidative stress, complement formation, inflammation, and cell survival networks.

About Stargardt Disease
Stargardt disease is a genetic eye disorder that causes retinal degeneration and vision loss. Stargardt disease is the most common form of inherited macular degeneration. The progressive vision loss associated with Stargardt disease is caused by the degeneration of photoreceptor cells in the central portion of the retina called the macula.

Decreased central vision due to loss of photoreceptors in the macula is the hallmark of Stargardt disease. Some peripheral vision is usually preserved. Stargardt disease typically develops during childhood or adolescence, but the age of onset and rate of progression can vary. The retinal pigment epithelium (RPE), a layer of cells supporting photoreceptors, is also affected in people with Stargardt disease.

About Ocugen, Inc.
Ocugen, Inc. is a pioneering biotechnology leader in gene therapies for blindness diseases. Our breakthrough modifier gene therapy platform has the potential to address significant unmet medical need for large patient populations through our gene-agnostic approach. Unlike traditional gene therapies and gene editing, Ocugen's modifier gene therapies address the entire disease—complex diseases that are potentially caused by imbalances in multiple gene networks. Currently we have programs in development for inherited retinal diseases and blindness diseases affecting millions across the globe, including retinitis pigmentosa, Stargardt disease, and geographic atrophy—late stage dry age-related macular degeneration. Discover more at www.ocugen.com and follow us X and LinkedIn.

Cautionary Note on Forward-Looking Statements

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995, including, but not limited to, statements regarding qualitative assessments of available data, potential benefits, expectations for ongoing clinical trials, anticipated regulatory filings and anticipated development timelines, which are subject to risks and uncertainties. We may, in some cases, use terms such as "predicts," "believes," "potential," "proposed," "continue," "estimates," "anticipates," "expects," "plans," "intends," "may," "could," "might," "will," "should," or other words that convey uncertainty of future events or outcomes to identify these forward-looking statements. Such statements are subject to numerous important factors, risks, and uncertainties that may cause actual events or results to differ materially from our current expectations, including, but not limited to, the risks that preliminary, interim and top-line clinical trial results may not be indicative of, and may differ from, final clinical data; the ability of OCU410ST to perform in humans in a manner consistent with nonclinical, preclinical or previous clinical study data; that unfavorable new clinical trial data may emerge in ongoing clinical trials or through further analyses of existing clinical trial data; that earlier non-clinical and clinical data and testing of may not be predictive of the results or success of later clinical trials; and that that clinical trial data are subject to differing interpretations and assessments, including by regulatory authorities. These and other risks and uncertainties are more fully described in our periodic filings with the Securities and Exchange Commission (SEC), including the risk factors described in the section entitled "Risk Factors" in the quarterly and annual reports that we file with the SEC. Any forward-looking statements that we make in this press release speak only as of the date of this press release. Except as required by law, we assume no obligation to update forward-looking statements contained in this press release whether as a result

of new information, future events, or otherwise, after the date of this press release.

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