Ocugen, Inc. Announces Positive Scientific Advice from the European Medicines Agency Related to the Approval Pathway for OCU410ST—Modifier Gene Therapy for Stargardt Disease

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MALVERN, Pa., Aug. 13, 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 Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) reviewed the study design, endpoints and planned statistical analysis of the ongoing pivotal confirmatory OCU410ST Phase 2/3 GARDian3 clinical trial for Stargardt disease and provided acceptability of a single U.S.-based trial for submission of a Marketing Authorization Application (MAA).

EMA provided this opinion based on safety and tolerability that OCU410ST demonstrated in the Phase 1 GARDian trial, including 48% slower lesion growth and statistically significant (p=0.031) and clinically meaningful improvement of nearly 2-line/9-letter gain in best corrected visual acuity (BCVA) at 12-month follow-up in evaluable treated eyes compared to untreated eyes. The Phase 2/3 study will enroll 51 participants diagnosed with Stargardt disease. Of these, 34 will receive a one-time subretinal injection of OCU410ST (200 µL at a concentration of 1.5×10^{11} vector genomes/mL) in the eye with poorer visual acuity, while 17 will be assigned to an untreated control group. The unique adaptive design of this trial includes a masked interim analysis of 24 subjects in the study (16 in treatment group and 8 in control group) at 8 months. The primary objective of the trial is to evaluate the reduction in atrophic lesion size. Key secondary endpoints include improvements in BCVA and low luminance visual acuity (LLVA), compared to controls. Data from the one-year follow-up will be used to support the Company's planned Biologics License Application (BLA) and MAA in the EU.

"This positive opinion endorses a single trial as the basis for both BLA and MAA submissions and brings us closer to providing a one-time, modifier gene therapy to approximately 100,000 Stargardt patients in the U.S. and Europe combined," said Dr. Shankar Musunuri, Chairman, CEO, and Co-founder of Ocugen. "We are very encouraged about the prospect of addressing the unmet medical need that exists for these patients who currently have no approved treatment options available to them."

The EMA opinion is an extremely favorable outcome, as it will potentially reduce the time and cost to gain marketing authorization in the EU. Alignment with the EMA follows recent important milestones for the OCU410ST program, including Rare Pediatric Disease Designation (RPDD) in May, IND clearance in June, and first patient dosing in July. With enrollment scheduled to be complete in the first quarter of 2026 the Company remains on track for a BLA filing in the first half of 2027, aligned with its goal of three BLAs in the next three years.

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 biotechnology company focused on discovering, developing, and commercializing novel gene therapies to address major blindness diseases and offer hope for patients across the globe. We are making an impact on patient's lives through courageous innovation—forging new scientific paths that harness our unique intellectual and human capital. Our breakthrough modifier gene therapy platform has the potential to address significant unmet medical need for large patient populations through our gene-agnostic approach. Discover more at www.ocugen.com and follow us on 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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