# Ocugen Announces Positive Opinion of EMA's Committee for Advanced Therapies for ATMP Classification for Novel Modifier Gene Therapy Candidate OCU410 for Geographic Atrophy and OCU410ST for Stargardt Disease

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MALVERN, Pa., March 03, 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 European Commission has provided a positive opinion from the European Medicines Agency's (EMA) Committee for Advanced Therapies (CAT) for OCU410 and OCU410ST Advanced Therapy Medicinal Product (ATMP) classification. OCU410 is a novel, multifunctional modifier gene therapy candidate being developed for the treatment of patients with vision loss due to geographic atrophy (GA)—an advanced stage of dry age-related macular degeneration (dAMD)—and OCU410ST is being developed for Stargardt disease due to *ABCA4*-related retinopathies.

GA affects 2-3 million people in the United States (U.S.) and Europe combined. There are two approved therapies in the U.S. that require frequent dosing (every month or every other month), however neither therapy has been approved in Europe. Stargardt disease affects 100,000 people in the U.S. and Europe combined, and there are no approved treatments available globally.

"Receiving ATMP classification for OCU410 and OCU410ST is a critical step to potentially address these severely unmet medical needs in the very near future," said Dr. Shankar Musunuri, Chairman, CEO, and Co-founder of Ocugen. "Dosing of Phase 2 in the ongoing OCU410 ArMaDa clinical trial is complete, and we are on track to initiate the Phase 3 clinical trial next year to pursue potential Marketing Authorization Application (MAA) and Biologics License Application (BLA) filings in 2028. Last week, the U.S. Food and Drug Administration (FDA) endorsed Ocugen's plan to move forward with a Phase 2/3 pivotal confirmatory clinical trial for OCU410ST, which can be the basis of BLA and potential MAA submissions in 2027."

ATMP classification is granted to medicines that can offer groundbreaking opportunities for the treatment of disease and accelerates the regulatory review timeline of this potential one-time gene therapy for life. Additionally, this classification allows Ocugen to interact with EMA more frequently for scientific advice and protocol assistance.

Preliminary 9-month data of OCU410 in GA patients demonstrated considerably slower lesion growth (44%) from baseline and clinically meaningful 2-line (10-letter) improvement in visual function (LLVA) in treated eyes compared to untreated eyes in the Phase 1 portion of the trial. Furthermore, a single subretinal OCU410 treatment preserves more retinal tissue around GA lesions of treated eyes at 9 months compared to published data on currently available GA therapies.

6-month data from Phase 1 of the OCU410ST GARDian clinical trial demonstrated considerably slower lesion growth (52%) from baseline in treated eyes versus untreated fellow eyes and clinically meaningful 2-line (10-letter) improvement in visual function (BCVA), which is statistically significant ($p=0.02$) in treated eyes. The Company plans to initiate the Phase 2/3 pivotal confirmatory clinical trial for OCU410ST by mid-2025.

"The novel modifier gene in OCU410 and OCU410ST targets all four pathways linked with dAMD and Stargardt and is delivered through a single subretinal injection as a one-and-done treatment," said Dr. Huma Qamar, Chief Medical Officer at Ocugen. "We are very pleased with the structural and functional outcomes demonstrated by both of these candidates, along with a stellar safety profile."

OCU410 and OCU410ST utilize an adeno-associated virus (AAV) platform for the retinal delivery of the *RORA* (ROR Related Orphan Receptor A) gene. The RORA protein plays an important role in lipid metabolism, reducing lipofuscin deposits and oxidative stress, and demonstrates an anti-inflammatory role as well as inhibiting the complement system in both *in vitro* and *in vivo* (animal model) studies.

**About Ocugen, Inc.**
Ocugen, Inc. is a biotechnology company focused on discovering, developing, and commercializing novel gene and cell therapies, biologics, and vaccines that improve health 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 treat multiple retinal diseases with a single product, and we are advancing research in infectious diseases to support public health and orthopedic diseases to address unmet medical needs. 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 OCU410 and 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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