# Ocugen, Inc. Announces Positive Scientific Advice from the European Medicines Agency Related to the Approval Pathway for OCU400—Modifier Gene Therapy for Broad Retinitis Pigmentosa Indication

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MALVERN, Pa., April 10, 2024 (GLOBE NEWSWIRE) -- Ocugen, Inc. (“Ocugen” or the “Company”) (NASDAQ: OCGN), a biotechnology company focused on discovering, developing, and commercializing novel gene and cell therapies, and vaccines, 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 pivotal OCU400 Phase 3 liMeliGhT clinical trial for retinitis pigmentosa (RP) and provided acceptability of the U.S.-based trial for submission of a Marketing Authorization Application (MAA).

EMA provided this opinion based on safety and tolerability of OCU400 demonstrated in the Phase 1/2 study. The Phase 3 liMeliGhT study will have a sample size of 150 participants primarily in the U.S.—one arm of 75 participants with *RHO* gene mutations and the other arm with 75 participants that are gene agnostic (representing multiple gene mutations associated with RP). In each arm, participants will be randomized 2:1 to the treatment group ($2.5 \times 10^{10}$ vg/eye of OCU400) and untreated control group, respectively.

The positive scientific advice from EMA is in alignment with U.S. FDA clearance of the IND amendment to initiate the Phase 3 liMeliGhT clinical trial of OCU400. OCU400 is the first gene therapy to enter Phase 3 with a broad RP indication. Previously, OCU400 received broad Orphan Drug Designation for RP and Leber congenital amaurosis in the EU.

“We are very grateful to EMA for their collaborative discussions and support in providing a gene-agnostic therapeutic option to RP patients with severe unmet medical need,” said Dr. Shankar Musunuri, Chairman, CEO and Co-founder of Ocugen. “This positive opinion is a critical step in providing our game-changing modifier gene therapies to patients globally.”

The EMA opinion is an extremely favorable outcome, as it will potentially reduce the time and cost to gain marketing authorization in the EU. With this milestone, OCU400 remains on track for 2026 BLA and MAA approval targets.

**About OCU400**
OCU400 is the Company’s gene-agnostic modifier gene therapy product based on NHR gene, *NR2E3*. *NR2E3* regulates diverse physiological functions within the retina—such as photoreceptor development and maintenance, metabolism, phototransduction, inflammation and cell survival networks. Through its drive functionality, OCU400 resets altered/affected cellular gene-networks and establishes homeostasis—a state of balance, which has the potential to improve retinal health and function in patients with inherited retinal diseases.

**About RP**
RP is a group of rare, genetic disorders that involve a breakdown and loss of cells in the retina, leading to vision loss and blindness. Currently, RP is associated with mutations in more than 100 genes.

There are no approved treatment options that slow or stop the progression of multiple forms of RP. Proposed treatments for RP include gene-replacement therapy, retinal implant devices, retinal transplantation, stem cells, vitamin therapy, and other pharmacological treatments. Current gene-replacement therapies are promising but are limited to treating just a single mutation. In addition, while gene therapies may provide a new functional gene, they do not necessarily eliminate the underlying genetic defect, which may still cause stress and toxic effects leading to retina degeneration. Therefore, the development of gene-specific replacement therapy is highly challenging, especially when multiple and unknown genes are involved. Thus, novel therapeutic approaches targeting broader RP disease in a gene agnostic manner offer greater hope for patients.

**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.

**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; 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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