Ocugen Announces Positive Opinion of European Medicines Agency's Committee for Advanced Therapies for Advanced Therapy Medicinal Product Classification for Modifier Gene Therapy Candidate OCU400 for Retinitis Pigmentosa

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MALVERN, Pa., Feb. 03, 2025 (GLOBE NEWSWIRE) -- Ocugen, Inc. (Ocugen or the Company) (NASDAQ: OCGN), a biotechnology company focused on discovering, developing, and commercializing novel gene and cell therapies, biologics, and vaccines, today announced that the European Commission has provided a positive opinion from the European Medicines Agency's (EMA) Committee for Advanced Therapies (CAT) for OCU400 Advanced Therapy Medicinal Product (ATMP) classification. OCU400 is the first gene therapy to enter Phase 3 with a broad retinitis pigmentosa (RP) indication.

"Receiving ATMP classification is another significant milestone toward bringing OCU400 to the market in Europe," said Dr. Shankar Musunuri, Chairman, CEO, and Co-founder of Ocugen. "This designation makes it possible to stay on track with our clinical and commercial strategy and potentially provide this novel modifier gene therapy candidate to all RP patients in the United States (U.S.) and Europe by 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 as the Company pursues Marketing Authorization Application (MAA) filing in 2026.

Underscoring the vital need for gene-agnostic treatments for diseases with multiple mutations such as RP, both the U.S. Food and Drug Administration (FDA) and EMA have acknowledged that the ongoing single, pivotal Phase 3 trial of OCU400 can suffice for Biologics License Application (BLA)/MAA submissions. Ocugen intends to file simultaneously in the U.S. and Europe upon completion of the Phase 3 trial.

The Phase 3 OCU400 liMeliGhT clinical trial is currently enrolling. The study has a sample size of 150 participants—one arm of 75 participants with *RHO* gene mutations and the other arm with 75 participants that are gene agnostic. In each arm, participants will be randomized 2:1 to the treatment group (2.5×10^{10} vg/eye of OCU400) and untreated control group, respectively. Patients eight years of age and older, with early through late-stage advancement of RP, are being recruited to participate in the liMeliGhT study.

"We are encouraged by the EMA's recognition of OCU400 as the Phase 3 liMeliGhT clinical trial advances," said Dr. Huma Qamar, Chief Medical Officer at Ocugen. "I look forward to working collaboratively with the EMA to address the unmet medical need that remains for nearly 98% of the RP patient population."

RP affects nearly 310,000 patients in the U.S., EU, and Canada. Currently, RP is associated with mutations in more than 100 genes and there are no approved treatment options that slow or stop the progression of multiple forms of RP.

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 Ocugen, Inc.
Ocugen, Inc. is a biotechnology company focused on discovering, developing, and commercializing novel gene and cell therapies 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 OCU400 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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