Ocugen, Inc. Announces FDA Approval of Expanded Access Program for Patients with Retinitis Pigmentosa

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MALVERN, Pa., Aug. 05, 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 it has received notification from FDA to begin its expanded access program (EAP) for the treatment of adult patients, aged 18 and older, with retinitis pigmentosa (RP) with OCU400—a modifier gene therapy product candidate.

“Each clinical milestone achieved by OCU400 brings us closer to providing a potential one-time treatment for life to patients living with RP,” said Dr. Shankar Musunuri, Chairman, CEO, and Co-founder of Ocugen. “With positive Phase 1/2 study data and an ongoing Phase 3 liMeliGhT (pronounced “limelight”) clinical trial, we now plan to work with clinicians, patients, and the RP community to provide access to OCU400 for eligible patients through our EAP. The EAP strengthens our commitment to serving RP patients—300,000 in the U.S. and Europe and 1.6 million globally.”

EAP allows patients who have unmet medical needs with serious or life-threatening conditions to access treatments outside of a clinical trial that are not yet approved by the FDA.

The OCU400 EAP is available for patients with early, intermediate to advanced RP with at least minimal retinal preservation who may benefit from the mechanism of action of OCU400 prior to approval of the Biologics License Application (BLA). Ocugen is actively dosing patients in the Phase 3 liMeliGhT clinical trial.

“RP patients with mutations in multiple genes currently have no therapeutic options. As a retinal surgeon, I am encouraged by the therapeutic potential of OCU400 to provide long-term benefit,” said Lejla Vajzovic, MD, FASRS, Director, Duke Surgical Vitreoretinal Fellowship Program, Associate Professor of Ophthalmology with Tenure, Adult and Pediatric Vitreoretinal Surgery and Disease, Duke University Eye Center, and Retina Scientific Advisory Board Chair of Ocugen. “The OCU400 EAP gives RP patients access to this novel modifier gene therapy outside of the ongoing Phase 3 study.”

“We are pleased to make OCU400 available to patients beyond our Phase 3 liMeliGhT clinical trial through this EAP,” said Dr. Huma Qamar, Ocugen’s Chief Medical Officer. "We are excited to expand our enrollment to include patients representing a diverse array of RP gene mutations. This program reflects our ongoing commitment to develop a safe and effective therapy for RP patients who may not have other treatment options.”

Ocugen previously announced that OCU400 has received orphan drug and Regenerative Medicine Advanced Therapy (RMAT) designations from FDA and that the European Medicines Agency (EMA) accepted the U.S.-based trial for submission of a Marketing Authorization Application (MAA). With the dosing of patients in the Phase 3 clinical trial program underway, OCU400 remains on track for targeted BLA and MAA approval in 2026.

About OCU400 EAP
The OCU400 EAP is a U.S.-only protocol for (1) eligible adult RP patients, 18 years and older, with early, intermediate to advanced disease with at least minimal retinal preservation, (2) patients who participated in the OCU400 Phase 1/2 study and who qualify for dosing in the contralateral eye, (3) patients who failed to meet inclusion criteria in the Phase 1/2 trial and ongoing Phase 3 liMeliGhT clinical trial who could benefit from OCU400, and (4) RP patients who can benefit from the mechanism of action of OCU400 prior to BLA approval.

Additional information on the OCU400 EAP will be available on www.clinicaltrials.gov.

About OCU400 Phase 3 (liMeliGhT) for RP
The Phase 3 liMeliGhT clinical trial, with a duration of one year, will have a sample size of 150 participants. One arm will include 75 participants with *RHO* gene mutations, and the other arm will include 75 participants who have mutations in other genes. Within each arm, participants will be randomized 2:1 to the treatment group (2.5×10^{10} vector genomes/eye of OCU400) and untreated control group, respectively. Patients eight years of age and older with early to late-stage RP are being recruited to participate in the liMeliGhT study.

About OCU400
OCU400 is the Company’s modifier gene therapy product based on a nuclear hormone receptor (NHR) gene called *NR2E3*. This gene regulates diverse physiological functions within the retina, such as photoreceptor development and maintenance, metabolism, phototransduction, inflammation, and cell survival. Retinal cells in RP patients have a dysfunctional gene network, and OCU400 resets this network to reestablish a healthy cellular homeostasis—which has the potential to improve vision in patients with RP.

About Modifier Gene Therapy
Modifier gene therapy is designed to fulfill unmet medical needs related to retinal diseases, including IRDs, such as RP, Leber congenital amaurosis (LCA) and Stargardt disease, as well as multifactorial diseases like dry age-related macular degeneration (dAMD). Our modifier gene therapy platform is based on the use of NHRs, master gene regulators, which have the potential to restore homeostasis — the basic biological processes in the retina. Unlike single-gene replacement therapies, which only target one genetic mutation, we believe that our modifier gene therapy platform, through its use of NHRs, represents a novel approach that has the potential to address multiple retinal diseases caused by mutations in multiple genes with one product, and to address complex diseases that are potentially caused by imbalances in multiple gene networks. Currently, Ocugen has three modifier

gene therapy programs in the clinic: OCU400, OCU410, and OCU410ST. In addition to the OCU400 Phase 3 liMeliGhT clinical trial, the OCU410 Phase 1/2 ArMaDa clinical trial for geographic atrophy (GA) secondary to dAMD and the OCU410ST Phase 1/2 GARDian clinical trial for Stargardt disease are currently underway. GA affects approximately two to three million people in the U.S. and EU combined and Stargardt disease affects nearly 100,000 people in the U.S. and EU combined.

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 patients' 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; 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.

Contact:
Tiffany Hamilton
Head of Corporate Communications
Tiffany.Hamilton@ocugen.com