Ocugen, Inc. Announces First Patient Dosed in Phase 3 liMeliGhT Clinical Trial for OCU400—First Gene Therapy in Phase 3 with a Broad Retinitis Pigmentosa Indication

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MALVERN, Pa., June 20, 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 first patient has been dosed in its Phase 3 liMeliGhT clinical trial for OCU400—a modifier gene therapy product candidate being developed for retinitis pigmentosa (RP).

“Each clinical milestone achieved by OCU400 brings us closer to providing a one-time treatment for life to patients living with RP,” said Dr. Shankar Musunuri, Chairman, CEO and Co-founder of Ocugen. “Dosing the first patient is especially significant and makes our dedication to serving RP patients—300,000 in the U.S. and Europe and 1.6 million worldwide—more tangible.”

The Phase 3 liMeliGhT clinical trial was informed by positive Phase 1/2 OCU400 data that suggests positive trends in Best-Corrected Visual Acuity (BCVA) and Multi-Luminance Mobility Testing (MLMT), and Low-Luminance Visual Acuity (LLVA) among treated eyes. 89% (16/18) of RP subjects demonstrated preservation or improvement in the treated eye either on BCVA or LLVA or MLMT scores from baseline. 80% (8/10) of *RHO* mutation subjects experienced either preservation or improvement in MLMT scores from baseline. 78% (14/18) of subjects demonstrated preservation or improvement in treated eyes in MLMT scores from baseline.

The Phase 3 study—with the duration of one year—will have 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 x 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.

Luminance Dependent Navigation Assessment (LDNA)—a more sensitive and specific measurement of function than MLMT used in previous Phase 3 clinical trials—is the primary endpoint for the study. The Phase 3 liMeliGhT study will focus on the proportion of responders, in treated and untreated groups, achieving an improvement of at least 2 Lux levels from baseline in the study eyes.

“Patients with RP associated 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 to RP patients,” 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. “OCU400 is a novel modifier gene therapy approach that could initiate a paradigm shift in the treatment of RP and to field of ophthalmology.”

“The current OCU400 Phase 3 study is very exciting and gives hope for thousands of individuals with RP,” said Benjamin Bakall, MD, PhD, Director of Clinical Research at Associated Retina Consultants (ARC) and Clinical Assistant Professor at University of Arizona, College of Medicine – Phoenix. “I am encouraged that we may have a potential treatment option to preserve or improve the vision in RP patients regardless of gene mutation, and very pleased that the first patient dosing in the Phase 3 liMeliGhT clinical trial was performed at ARC.”

“We are grateful for our continued collaboration with Dr. Bakall and the team at ARC,” said Dr. Huma Qamar, Chief Medical Officer of Ocugen. "We are excited to expand our enrollment to include more centers and patients representing a diverse array of RP gene mutations, which will be a validation of this novel gene therapy platform. We will provide updates as our progress continues."

Ocugen previously announced that OCU400 has received orphan drug and RMAT designations from the FDA and that the EMA provided acceptability of the U.S.-based trial for submission of a Marketing Authorization Application (MAA). With the first dosing of the Phase 3 clinical trial, OCU400 remains on track for the 2026 BLA and MAA approval targets.

About OCU400
OCU400 is the Company’s gene-agnostic modifier gene therapy product based on nuclear hormone receptor (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 RP.

About Modifier Gene Therapy
Modifier gene therapy is designed to fulfill unmet medical needs related to retinal diseases, including IRDs, such as RP, 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 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; 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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