Data and Safety Monitoring Board Approves Simultaneous Enrollment in Cohort 3 and Phase 2 Initiation in OCU410 ArMaDa study for Geographic Atrophy

May 31, 2024

- *Established Medium Dose as Safe and Tolerable Dose in Current OCU410 Clinical Trial*
- *DSMB Recommends Continuing with High-Dose Cohort Dosing with Concurrent Phase 2 Dosing*

MALVERN, Pa., May 31, 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 a positive outcome of the Data and Safety Monitoring Board (DSMB) Review for its Phase 1/2 ArMaDa clinical trial for OCU410 (*AAV5-hRORA*)—a modifier gene therapy candidate being developed for geographic atrophy (GA), an advanced stage of dry age-related macular degeneration (dAMD). GA affects approximately 2-3 million people in the U.S. and Europe combined.

Six subjects with GA were dosed in the Phase 1/2 clinical trial to date—three subjects were dosed with the low dose and three subjects were dosed with the medium dose. An additional three patients will be dosed with the high dose of OCU410 in the dose-escalation phase.

“The DSMB has recommended to proceed with dosing subsequent GA subjects with the high dose of OCU410 in the dose-expansion phase of the study and concurrently initiate Phase 2 dosing,” said Dr. Peter Chang, MD, FACS, DSMB Chair for the OCU410 clinical trial. “No serious adverse events (SAEs) related to OCU410 have been reported to date in both low- and medium-dose cohorts. I believe that this marks a critical next step towards determining the maximum tolerated dose for OCU410 and is an important milestone for its clinical development.”

"We are delighted to report a second positive DSMB recommendation for the treatment of GA, which significantly builds on the favorable safety and tolerability profile exhibited by OCU410," said Huma Qamar, MD, MPH, CMI, Chief Medical Officer of Ocugen. "We are very enthusiastic about the potential of OCU410 as a potential one-time treatment for GA with a single sub-retinal injection. The currently approved treatments for GA target only the complement pathway and require approximately 6-12 intravitreal injections annually. OCU410 addresses multiple pathways causing dAMD, including complement, lipid metabolism, inflammation, and oxidative stress, providing long-term benefit to patients."

The ArMaDa clinical trial will assess the safety and efficacy of unilateral subretinal administration of OCU410 in subjects with GA and will be conducted in two phases. Phase 1 is a multicenter, open-label, dose-ranging study consisting of three dose levels [low dose (2.5×10^{10} vg/mL), medium dose (5×10^{10} vg/mL), and high dose (1.5×10^{11} vg/mL)]. Phase 2 is a randomized, outcome accessor-blinded, dose-expansion study in which subjects will be randomized in a 1:1:1 ratio to either one of two OCU410 treatment groups or to an untreated control group.

About dAMD and GA
dAMD affects approximately 10 million Americans and more than 266 million people worldwide. It is characterized by the thinning of the macula. The macula is the part of the retina responsible for clear vision in one’s direct line of sight. dAMD involves the slow deterioration of the retina with submacular drusen (small white or yellow dots on the retina), atrophy, loss of macular function and central vision impairment. dAMD accounts for 85-90% of the total AMD population.

About OCU410
OCU410 utilizes an AAV delivery 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 in-vitro and in-vivo (animal model) studies. These results demonstrate the ability for OCU410 to target multiple pathways linked with dAMD pathophysiology. Ocugen is developing AAV-RORA as a one-time gene therapy for the treatment of GA.

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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