Ocugen Announces Positive Data and Safety Monitoring Board Review and Initiation of Enrollment in Medium Dose for OCU410—a Modifier Gene Therapy—in Phase 1/2 ArMaDa Study for Geographic Atrophy

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- *Established Low Dose as Safe and Tolerable Dose in Current OCU410 Clinical Trial*
- *DSMB Approval to Proceed with Medium Dose Cohort Dosing*

Malvern, Pa., April 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 the Data and Safety Monitoring Board (DSMB) for the Phase 1/2 ArMaDa clinical trial for OCU410 recently convened and approved to proceed dosing with the medium dose of OCU410 in the dose-escalation phase of the study.

Three subjects with geographic atrophy (GA) were dosed in the Phase 1/2 clinical trial to date. An additional three subjects will be dosed with the medium dose (Cohort 2) and three patients with the high dose (Cohort 3) of OCU410 in the dose-escalation phase.

“The DSMB has recommended moving forward to medium dose for dosing subjects with GA,” 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. I believe that this marks a critical next step towards determining the optimal dosing regimen and an important milestone for the clinical development of OCU410.”

“The positive DSMB review for the first modifier gene therapy for GA significantly builds on the favorable safety and tolerability profile exhibited by OCU410,” said Huma Qamar, M.D., MPH, Chief Medical Officer of Ocugen. “We are very enthusiastic about the potential of OCU410 as a one-time treatment for life with a single sub-retinal injection.”

Currently approved products to treat GA have significant limitations, as they require multiple injections per year and only target one pathway contributing to GA. OCU410 regulates multiple pathways involved with the disease including: lipid metabolism, inflammation, oxidative stress, and membrane attack complex (complement).

The ArMaDa clinical trial will assess the safety 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.

Ocugen is committed to finding solutions for inherited retinal diseases as well as blindness diseases affecting millions. GA is an advanced form of dry age-related macular degeneration (dAMD) and affects approximately 1 million people in the United States.

About dAMD
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, 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. 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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