Data and Safety Monitoring Board Reviews Interim Safety Data of Phase 2 Subjects of OCU410 ArMaDa Clinical Trial for Geographic Atrophy Secondary to Dry Age-Related Macular Degeneration

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- *OCU410 has a very favorable safety and tolerability profile*
- *No serious adverse events related to the study drug have been reported, such as exudation, infectious endophthalmitis, intraocular Inflammation, anterior ischemic optic neuropathy, or vasculitis*

MALVERN, Pa., Dec. 19, 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, biologics, and vaccines, today announced that the Data and Safety Monitoring Board (DSMB) for the OCU410 ArMaDa clinical trial recently convened and approved continuation of the second phase of the Phase 1/2 study. OCU410 (AAV5-h*RORA*) is a novel modifier gene therapy candidate being developed for geographic atrophy (GA) secondary to dry age-related macular degeneration (dAMD).

“The DSMB assessed data on 15 subjects from Phase 2. Initial data indicates that OCU410 appears to be safe and well-tolerated,” said Peter Chang, MD, FACS, Co-President and Partner of the Massachusetts Eye Research and Surgery Institution (MERSI). “No serious adverse events (SAEs) related to OCU410 have been reported to date.”

The ArMaDa clinical trial will assess the safety and efficacy of unilateral subretinal administration of OCU410 in subjects with GA. Phase 2 is an ongoing, randomized, outcome assessor-blinded, dose-expansion study in which 45 subjects are randomized in a 1:1:1 ratio to either one of two OCU410 treatment groups (5×10^{10} vg/mL or 1.5×10^{11} vg/mL) or an untreated control group.

“Currently approved treatments for GA require 6-12 intravitreal injections annually and frequent injections are a burden on patients and caregivers,” said Huma Qamar, MD, MPH, CMI, Chief Medical Officer of Ocugen. “We are very enthusiastic about the potential of OCU410 to serve as a game-changing, one-time treatment for life for patients with GA.”

Positive preliminary efficacy and safety data from the Phase 1 dose-escalation portion of the ArMaDa clinical trial demonstrated: no drug-related serious adverse events, reduced lesion growth, preservation of retinal tissue, and—most importantly—there was a positive effect on the functional visual measure of low luminance visual acuity (LLVA).

dAMD is a multifactorial disease involving genetic and environmental factors that is one of the world’s leading causes of blindness in people aged 50 years and older. Four cellular pathways drive the pathology of dry AMD: lipid metabolism, inflammation, oxidative stress, and complement. Currently approved therapies target only the latter, while OCU410 addresses all four and thereby helps reestablish retinal homeostasis.

The ArMaDa clinical trial is currently being performed at 13 leading retinal surgery centers across the U.S. Dosing in the OCU410 ArMaDa clinical trial will be completed in early 2025 and the Company will continue to provide 9- and 12-month efficacy updates from Phase 1.

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* (RAR 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 *in vitro* and *in vivo* (animal model) studies. These results demonstrate the ability of OCU410 to target multiple pathways linked with dAMD pathophysiology. Ocugen is developing AAV5-h*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, strategy, business plans and objectives for Ocugen’s clinical programs, plans and timelines for the preclinical and clinical development of Ocugen’s product candidates, including the therapeutic potential, clinical benefits and safety thereof, expectations regarding timing, success and data announcements of current ongoing preclinical and clinical trials, the ability to initiate new clinical programs; 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 annual and 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 Communications
Tiffany.Hamilton@ocugen.com