Ocugen Announces Topline 12-month Data from Phase 2 ArMaDa Clinical Trial Evaluating OCU410 Modifier Gene Therapy for Geographic Atrophy Secondary to Dry Age-Related Macular Degeneration

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- *Optimal dose intended for Phase 3 demonstrates statistically significant reduction in lesion growth (31%) versus control at 12 months (p< 0.05)*
- *Potential 2X treatment benefit compared to 15% and 22% reductions reported for currently approved therapies at 12 and 24 months, respectively*
- *No serious adverse events and no adverse events of special interest related to OCU410 reported to date*

MALVERN, Pa., March 24, 2026 (GLOBE NEWSWIRE) -- Ocugen, Inc. (Ocugen or the Company) (NASDAQ: OCGN), a pioneering biotechnology leader in gene therapies for blindness diseases, today announced positive 12-month data from the Phase 2 ArMaDa clinical trial evaluating OCU410 (AAV5-RORA), its novel modifier gene therapy for geographic atrophy (GA) secondary to dry age-related macular degeneration (dAMD). The global prevalence of dAMD is 266 million worldwide, and GA affects approximately 2-3 million people in the United States (U.S.) and Europe. Importantly, this number is expected to increase significantly as populations age.

There are limited options for patients with dAMD in the U.S. and current therapies require 6–12 injections per year indefinitely, leading to substantial burden and significant dropout rates in real-world practice. Outside of the U.S., there are no approved products available.

Key findings from Phase 2 include:

- 31% reduction in lesion growth in the optimal dose (medium) group compared to control (p< 0.05)
- 27% slower rate of ellipsoid zone (EZ) loss compared to control, indicating structural preservation of photoreceptors, which correlates with visual function
- 55% of treated patients demonstrated ≥30% lesion size reduction vs. control
- Subgroup analysis (subjects with baseline GA lesions ≥5 mm^2 and ≤17.5 mm^2) showed 33% reduction in lesion growth compared to control in medium dose OCU410 with similar reductions in the high dose group

The Phase 2 clinical trial builds directly on the clean safety profile observed in Phase 1 with no OCU410-related serious adverse events observed and no cases of endophthalmitis, retinal detachment, vasculitis, choroidal neovascularization, or ischemic optic neuropathy reported to date.

GA is a multifactorial disease with a complex etiology that involves genetic and environmental factors. The current treatment options for GA in the U.S. are limited to those targeting a single mechanism—the complement pathway. By contrast, OCU410 is a first-in-class RORA-based gene therapy designed to support central retina and photoreceptor integrity through a multi-pathway mechanism—targeting drusen, inflammation, oxidative stress, and complement activation.

“We have confirmed robust treatment effect from a well-controlled Phase 2 trial of a genetic medicine for GA. Now we can move on to Phase 3 with a high degree of confidence,” said Dr. Shankar Musunuri, Chairman, CEO, and Co-founder of Ocugen. “This moves us one step closer to bringing a transformative one-time treatment to GA patients globally who are desperately seeking rescue from vision loss.”

“Our Phase 2 data consistently demonstrates statistically significant reduction of GA lesion growth after treatment with OCU410 optimal dose, and we continue to benchmark these results against natural history data to contextualize the magnitude of effect,” said Huma Qamar, MD, MPH, CMI, Chief Medical Officer of Ocugen. “We are incorporating these learnings into an anticipated Phase 3 pivotal confirmatory trial with up to 300 subjects and an adaptive design powered at over 95%.”

“There remains a considerable unmet need in treating patients with GA and I am encouraged by the various analyses of the Phase 2 OCU410 data,” 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 Chair, Ocugen Retina Scientific Advisory Board. “In addition to the strong efficacy and safety data, OCU410 has the potential to eliminate the chronic treatment burden associated with monthly or every-other-month intravitreal injections and to reduce treatment attrition driven by patient fatigue.”

In the Phase 2 study, the safety and efficacy of OCU410 in patients with GA secondary to dAMD are being assessed. Fifty-one (51) patients aged 50 years and older with GA lesions within the foveal or non-foveal region were randomized 1:1:1 to receive a single subretinal administration of OCU410 at a medium dose of 1×10^{10} vector genomes per eye, a high dose of 3×10^{10} vector genomes per eye, or no treatment in the control group; each injection volume was 200 microliters. Of note, choroidal neovascularization in the fellow eye was not exclusionary, and patients with prior exposure to pegcetacoplan or avacincaptad pegol were eligible following a three-month washout.

The primary endpoint was change in GA lesion size at 12 months, measured in square millimeters by fundus autofluorescence, an FDA-accepted structural endpoint used in recent GA registration trials. Exploratory endpoints included EZ preservation on OCT a key biomarker for photoreceptor

integrity, which correlates with visual function.

Ocugen plans to initiate the OCU410 Phase 3 registrational trial in the third quarter of 2026 in line with the Company's goal of three BLA filings in three years.

About dAMD and Geographic Atrophy
Geographic atrophy is an advanced form of dAMD characterized by progressive degeneration of the macula, leading to irreversible central vision loss. Millions of patients worldwide are affected by GA, with a particularly high burden in aging populations in the United States and Europe. Despite recent approvals, treatment options remain limited and require chronic intravitreal injections, underscoring the need for innovative, durable therapies that address multiple disease mechanisms. dAMD affects approximately 10 million Americans and more than 266 million people worldwide. It is characterized by the thinning of the macula, the portion 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 all AMD cases.

About OCU410
OCU410 is an investigational, subretinal injection, AAV5-based gene therapy that delivers RORA (retinoid-related orphan receptor alpha), a nuclear receptor that regulates key pathways involved in retinal homeostasis, including oxidative stress response, complement regulation, inflammation, and lipid metabolism. OCU410 is being developed as a one-time gene therapy for patients with GA secondary to dry AMD. OCU410 has received Advanced Therapy Medicinal Product (ATMP) classification from the European Medicines Agency.

About Ocugen, Inc.
Ocugen, Inc. is a biotechnology company focused on discovering, developing, and commercializing novel gene therapies to address major blindness diseases 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 address significant unmet medical need for large patient populations through our gene-agnostic approach. 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; the ability of OCU410 to perform in humans in a manner consistent with nonclinical, preclinical or previous clinical study 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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