Ocugen Announces Positive Preliminary Phase 2 Data from OCU410 Modifier Gene Therapy for Geographic Atrophy Secondary to Dry Age-Related Macular Degeneration

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- *Phase 2 (~50% of patients evaluated to date at 12 months) shows 46% lesion growth reduction vs. control*
- *There are no OCU410-related serious adverse events reported across the Phase 1 and Phase 2 clinical trials to date*

MALVERN, Pa., Jan. 15, 2026 (GLOBE NEWSWIRE) -- Ocugen, Inc. (Ocugen or the Company) (NASDAQ: OCGN), a pioneering biotechnology leader in gene therapies for blindness diseases, today announced positive preliminary 12-month data (~50% of patients evaluated to date) 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 – the late stage of dAMD – affects approximately 2-3 million people in the United States (U.S.) and Europe.

There are limited options for patients with dAMD in the U.S. and current therapies involve frequent (monthly or every other month) injections and have unwanted side effects that can affect vision. Outside of the U.S., there are no approved products available, leaving approximately 2 million patients in Europe without a treatment option.

Key findings from Phase 2 include:

- 46% lesion growth reduction (medium + high dose vs. control; p=0.015; N=23) at 12 months
- Medium dose achieved 54% lesion reduction (p=0.02; N=10) vs. high dose 36% (p=0.05; N=8) compared to control
- 50% responder rate with patients achieving >50% lesion size reduction vs. control
- Subgroup (N=14, subjects with ≥7.5 mm^2 at baseline) showed 57% greater reduction in lesion size compared to control

New findings from Phase 1 (N=9) include:

- In evaluable subjects (N=7) ellipsoid zone (EZ) loss was 60% slower in OCU410-treated eyes compared to untreated fellow eyes at 12 months
- EZ-RPE complex loss reduced in treated eyes versus fellow eyes, demonstrating photoreceptor + RPE preservation

In both the Phase 1 and Phase 2 clinical trials no OCU410-related serious adverse events were observed and no cases of endophthalmitis, retinal detachment, vasculitis, choroidal neovascularization, or optic ischemic neuropathy have been 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—requiring frequent intravitreal injections, either monthly or every other month. By contrast, OCU410 is a multifunctional modifier gene therapy, which targets multiple pathways associated with GA.

"The OCU410 Phase 1 and Phase 2 results mark a pivotal moment for Ocugen's modifier gene therapy platform and GA patients worldwide," said Dr. Shankar Musunuri, Chairman, CEO, and Co-founder of Ocugen. "Delivering 60% slower EZ loss in Phase 1 and 46% lesion growth reduction in the Phase 2 preliminary analysis demonstrates the capability of our multi-pathway RORA approach. We look forward to reporting full data from the OCU410 Phase 2 clinical trial later this quarter and initiating Phase 3 in 2026."

"The clinical development journey of OCU410 has been remarkable," said Dr. Huma Qamar, Chief Medical Officer of Ocugen. "Our Phase 2 randomized trial delivered robust anatomic efficacy that was statistically significant across multiple analyses. Critically, our safety data across 60 patients has shown no drug-related serious adverse events, no inflammation signals, and no injection complications to date, supporting a favorable risk-benefit profile."

"As a practicing retinal specialist, OCU410's clinical profile is genuinely exciting for geographic atrophy patients—including a reduction in ellipsoid zone loss observed in Phase 1, which may serve as a potential marker of retinal health, and a reduction in lesion growth seen in Phase 2," said Lejla Vajzovic, MD, FASRS, Director, Duke Surgical Vitreoretinal Fellowship Program, Professor of Ophthalmology with Tenure, Adult and Pediatric Vitreoretinal Surgery and Disease, Duke University Eye Center, and Retina Scientific Advisory Board Chair of Ocugen. "With these promising results, I believe OCU410 has the potential to set a new standard of care with a single treatment for life."

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 were randomized 1:1:1 into either of two treatment groups (medium or high dose) or a control group. In the treatment groups, subjects received a single subretinal 200-µL administration of 5 x 10^{10} vector genomes (vg)/mL (medium dose) or 1.5 x 10^{11} vg/mL (high dose), while the control group remained untreated. The Company remains on track for a Biologics License Application (BLA) filing for OCU410 in 2028, aligned with its strategy to advance three regulatory submissions for marketing authorization 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, intravitreally administered, 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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