Ocugen Announces First Patient Dosed in Phase 3 Registrational Trial of OCU410 Modifier Gene Therapy for Geographic Atrophy Secondary to Dry Age-Related Macular Degeneration

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- The single global Phase 3 trial, ArMaDa3 (NCT07770828), is the first pivotal gene therapy trial in geographic atrophy (GA)
- U.S. Food and Drug Administration (FDA) granted OCU410 Regenerative Medicine Advanced Therapy (RMAT) designation, providing enhanced agency engagement throughout development and eligibility for accelerated approval and priority review
- Phase 3 design fully aligned with FDA; Biologics License Application (BLA) filing anticipated in 2028
- OCU410 is designed as a one-time subretinal gene therapy that addresses multiple disease pathways, offering a differentiated approach from approved complement inhibitors in the U.S. which address individual disease pathways and require ongoing intravitreal injections

MALVERN, Pa., Sept. 01, 2026 (GLOBE NEWSWIRE) -- **Ocugen**, **Inc.** ("Ocugen" or the "Company") (NASDAQ: OCGN), a pioneering biotechnology leader in gene therapies for blindness diseases, today announced that the first patient was dosed in the global Phase 3 registrational trial of OCU410 (AAV5-hRORA), its first-in-class modifier gene therapy candidate for GA secondary to dry age-related macular degeneration (dAMD). The Company also highlighted the recent FDA RMAT designation for OCU410, which supports a potential accelerated development and review pathway for the program.

"Dosing the first patient in our global Phase 3 trial, just weeks after receiving RMAT designation, marks a defining moment for the OCU410 program – and for the millions of people living with geographic atrophy. Outside the U.S., there are currently no approved treatments for GA, while in the U.S., available treatment options address only one of the four disease pathways and require ongoing, repeated eye injections," said Dr. Shankar Musunuri, Chairman, Chief Executive Officer, and Co-Founder of Ocugen. "This is our third modifier gene therapy program to advance into late-stage development, demonstrating the strength of our platform and our vision for potentially delivering a one-time treatment for life."

The initiation of dosing follows the successful completion of a Type B End-of-Phase 2 (EOP2) meeting with FDA's Center for Biologics Evaluation and Research (CBER) in July 2026, resulting in alignment on all critical Phase 3 design elements, including primary and secondary endpoints, dose, adaptive design, and a single pivotal trial pathway to support a BLA.

"We are entering a global single Phase 3 with a well-defined program: a dose validated in a randomized, controlled Phase 2 study; an FDA-endorsed primary endpoint measuring the rate of lesion growth; and a secondary endpoint assessing functional vision," said Mohamed Genead, MD, Chief Medical Officer of Ocugen. "The Phase 3 program builds on compelling 12-month Phase 2 data, which demonstrated a statistically significant 31% reduction in lesion growth with the optimal dose compared with control following a single subretinal injection, along with concordant preservation of the ellipsoid zone and no drug-related serious adverse events (SAEs) or adverse events of special interest (AESIs)."

OCU410 delivers the human *retinoid-related orphan receptor alpha (RORA)* modifier gene via a single subretinal injection of an AAV5 vector. Unlike therapies targeting a single pathway, OCU410 is designed to simultaneously address multiple pathophysiological drivers of GA-complement overactivation, chronic inflammation, oxidative stress, and lipid dysregulation. GA affects approximately 2–3 million people in the U.S. and Europe and is a leading cause of irreversible central vision loss in older adults, with prevalence expected to rise as the population ages.

According to study investigator Victor Gonzalez, MD, "Patients with geographic atrophy continue to face irreversible structural and functional loss of the retina, along with limited treatment options. The OCU410 Phase 3 study provides an important opportunity to evaluate a novel, potential one-time gene therapy approach that could lessen the burden of current treatments in the U.S., which require patients to undergo multiple injections every year."

Global Phase 3 Registrational Trial Design
The Phase 3 trial is a global, multicenter, randomized, controlled study enrolling 237 subjects with GA secondary to dAMD, randomized 2:1 to a single 200 µL subretinal injection of OCU410 (5×10^{10} vg/mL) or an untreated control arm, with sites in the United States, Canada, Europe, and Latin America.

- **Primary endpoint:** Rate of change of square root-transformed GA lesion area (√mm²/year) by fundus autofluorescence (FAF) at baseline, Month 4, Month 8, and Month 12, analyzed by MMRM.
- **Secondary endpoints:** Proportion of subjects with Low-Luminance Visual Acuity (LLVA) loss ≥15 ETDRS letters at two consecutive visits through Month 12, providing a functional vision anchor to the primary anatomic endpoint; and rate of change of ellipsoid zone (EZ) area loss by SD-OCT.

- **Regulatory path:** A single, adequate and well-controlled Phase 3 trial, aligned with FDA feedback, is intended to support a BLA filing anticipated in 2028. Discussions are ongoing with the European Medicines Agency (EMA) regarding alignment to potentially support a marketing authorization application (MAA) in Europe with this single Phase 3 trial.

RMAT Designation: Regulatory and Strategic Significance
On July 29, 2026, FDA granted RMAT designation to OCU410 based on Phase 2 clinical data demonstrating clinically meaningful efficacy and a favorable safety profile, with no serious adverse events related to OCU410 reported. RMAT designation is granted to regenerative medicine therapies intended to treat serious or life-threatening conditions where preliminary clinical evidence indicates the potential to address an unmet medical need.

For the OCU410 program, RMAT designation provides:

- **Eligibility for accelerated approval and priority review**, which may compress the time from BLA submission to potential market entry.
- **All benefits of Breakthrough Therapy designation**, including intensive FDA guidance on efficient development and organizational commitment involving senior FDA leadership.
- **Early and frequent FDA interactions** on the use of surrogate and intermediate clinical endpoints reasonably likely to predict long-term clinical benefit-directly relevant to OCU410's FAF-based anatomic primary endpoint.
- **Potential flexibility in satisfying post-approval requirements**, including through expanded patient registries or real-world evidence.

Taken together with FDA alignment on the Phase 3 design and the initiation of dosing, RMAT designation further de-risks the regulatory pathway for OCU410 and reinforces the differentiation of a one-time, multi-pathway gene therapy in a GA market currently served only by chronically administered intravitreal complement inhibitors.

Supporting Phase 2 ArMaDa Data
The Phase 3 trial and the RMAT designation are supported by 12-month data from the Phase 2 ArMaDa trial (NCT06018558), a multicenter, randomized, controlled study of 51 subjects with GA secondary to dAMD.

- **Lesion growth (FAF): 31% reduction** in GA lesion area growth rate in the medium dose group versus control at 12 months ($p < 0.05$) in the pivotal phase 3 population (lesion size of ≥2.5 mm^2 and ≤17.5 mm^2), a potential 2× treatment benefit relative to the 15% and 22% reductions reported for currently approved therapies in the U.S. at 12 and 24 months, respectively.
- **EZ preservation (SD-OCT): 27% reduction** in ellipsoid zone area loss in the medium dose group versus control, a structural correlate of visual function.
- **Responder analysis:** In the medium dose group, approximately 20% of treated subjects showed no disease progression; 75% demonstrated >30% reduction in lesion growth at 12 months.
- **Safety:** No OCU410-related serious adverse events (SAEs) or adverse events of special interest (AESIs) reported to date.

About OCU410
OCU410 (AAV5-hRORA) is Ocugen's investigational first-in-class modifier gene therapy, delivering the RORA gene via a single unilateral subretinal injection to regulate complement activation, neuroinflammation, oxidative stress, and lipid metabolism – multiple pathways implicated in the pathogenesis of GA. OCU410 has received RMAT designation from the FDA and Advanced Therapy Medicinal Product classification from the European Medicines Agency's Committee for Advanced Therapies.

About Ocugen, Inc.
Ocugen, Inc. is a pioneering biotechnology company developing gene therapies for blindness diseases. The Company's breakthrough modifier gene therapy platform has the potential to address significant unmet medical needs across large patient populations through a gene-agnostic approach. Unlike traditional gene therapies and gene-editing technologies that target a single gene mutation, Ocugen's modifier gene therapies are designed to address the underlying disease biology by restoring balance across multiple gene networks. The Company is currently advancing programs for inherited retinal diseases and other causes of blindness that affect millions worldwide, including retinitis pigmentosa, Stargardt disease, and geographic atrophy, an advanced form of dry age-related macular degeneration. Discover more at www.ocugen.com and follow us on LinkedIn and X.

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 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, including the timing of enrollment and data readouts, 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, statements regarding potential market size and commercial possibilities of Ocugen's product candidates, 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 receipt of RMAT designation may not lead to faster development or accelerated regulatory review or approval; 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 may not be predictive of the results or success of later clinical trials; and 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 quarterly 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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