Ocugen Announces Early Completion of Dosing in Phase 2/3 Pivotal Confirmatory Trial of OCU410ST for Stargardt Disease

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- GARDian3 trial enrollment and dosing completed (N=63) in less than nine months
- Topline results expected in 2Q27 with BLA to follow by mid-2027
- OCU410ST represents a potential first-in-class, one-time modifier gene therapy for all *ABCA4*-associated retinopathies

MALVERN, Pa., April 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 dosing has been successfully completed ahead of schedule in the Phase 2/3 GARDian3 pivotal confirmatory clinical trial for OCU410ST (AAV5-hRORA)—a modifier gene therapy candidate developed for all Stargardt disease (*ABCA4*-associated retinopathies).

“This enrollment milestone for a pivotal trial underscores the tremendous progress our team is making toward bringing a transformative therapy to people living with multiple *ABCA4*-related gene mutations including Stargardt disease,” said Dr. Shankar Musunuri, Chairman, Chief Executive Officer, and Co-founder of Ocugen. “The efficient and accelerated execution of this trial reflects the strong engagement of investigators and patients. It reinforces our confidence in OCU410ST as a potential one-time treatment option for all Stargardt patients who are desperately seeking rescue from blindness with no approved therapies to date.”

"I am encouraged by the enthusiastic response and rapid enrollment in the GARDian3 registrational clinical trial for Stargardt disease—a devastating pediatric-onset retinal disorder affecting approximately 100,000 patients in the U.S. and Europe," said Dr. Huma Qamar, Chief Medical Officer of Ocugen. “Our trial encompasses pediatric to adult, and early to advanced stage subjects to address critical unmet medical need.”

“As a treating retina specialist, I see how the natural history of Stargardt disease leads to relentless enlargement of atrophic lesions and gradual loss of central visual acuity, often at a young age," said Christine Kay, MD, Vitreo Retinal Associates, Florida and a principal investigator in the GARDian3 trial. "The opportunity to intervene at an early stage of disease with a one-time subretinal gene therapy like OCU410ST that can potentially slow lesion growth, preserve visual function over time, and save vision before irreversible damage represents an exciting and much needed shift from watching patients decline to proactively altering the course of their disease."

GARDian3 is a multicenter, randomized, masked, pivotal Phase 2/3 confirmatory study designed to evaluate the efficacy and safety of OCU410ST in patients with all mutations of Stargardt disease. OCU410ST is administered as a single subretinal injection, leveraging Ocugen’s AAV5-based modifier gene therapy platform to provide durable expression of hRORA in the retina with the goal of slowing or halting progressive macular degeneration and preserving visual function.

The Phase 2/3 study enrolled 63 participants diagnosed with Stargardt disease. Subjects randomized to treatment group received a one-time subretinal injection of OCU410ST (3×10^{10} vector genomes/eye) in the eye with poorer visual acuity, while untreated control group did not receive any treatment. The primary objective of the trial is to evaluate the reduction in atrophic lesion size at 12 months. Key secondary endpoints include improvements in best corrected visual acuity (BCVA) and low luminance visual acuity (LLVA), compared to controls. Observational endpoints include preservation of Ellipsoid Zone (EZ) that correlates to visual function. While demonstrating functional benefit via visual acuity within 12 months can be challenging due to the disease’s natural history, it is believed that preservation of EZ will serve as a meaningful and early indicator of therapeutic benefit.

Interim analysis will be performed in the third quarter of 2026 when 24 subjects complete the 8-month follow-up visit post-OCU410ST treatment. Data from the one-year follow-up will be used to support the company’s planned Biologics License Application (BLA).

OCU410ST maintains a favorable safety and tolerability profile with no serious adverse events or adverse events of special interest, including ischemic optic neuropathy, vasculitis, intraocular inflammation, endophthalmitis or choroidal neovascularization.

The OCU410ST Phase 2/3 pivotal confirmatory trial represents Ocugen’s second late-stage clinical program. Ocugen plans to submit the BLA for OCU410ST mid-2027 in alignment with its strategic goal of filing three BLAs by 2028.

About OCU410ST
OCU410ST utilizes an AAV5 delivery platform to deliver the *RORA* (RAR-Related Orphan Receptor A) gene to the retina. By restoring nuclear hormone receptor signaling, OCU410ST addresses pathophysiological pathways linked to Stargardt disease, including lipofuscin formation, oxidative stress, complement activation, inflammation, and photoreceptor survival networks independent of the underlying *ABCA4* genotype.

In a 12-month Phase 1 (GARDian 1) trial, evaluable treated eyes showed a 54% reduction in atrophic lesion growth versus untreated fellow eyes, with slower lesion expansion and improvement in visual acuity among evaluable patients. Treated eyes gained an average of 6 letters in BCVA, while untreated fellow eyes declined by 1.5 letters, and all treated eyes either stabilized or improved in visual acuity. In evaluable subjects ellipsoid zone (EZ) loss rate was 116% slower in OCU410ST-treated eyes vs untreated fellow eyes at 12 months. Data indicates preservation or stabilization of

photoreceptor integrity in treated eyes. No drug-related serious adverse events or adverse events of special interest were observed.

About Stargardt Disease
Stargardt disease type 1 is a genetic eye disorder caused by biallelic mutations in the *ABCA4* gene. This condition leads to progressive macular degeneration, with onset typically occurring during childhood or adolescence. Affected patients experience progressive central vision loss while peripheral vision is usually preserved. There are currently no FDA-approved treatments for this orphan indication.

About Ocugen, Inc.
Ocugen, Inc. is a pioneering biotechnology leader in gene therapies for blindness diseases. Our breakthrough modifier gene therapy platform has the potential to address significant unmet medical need for large patient populations through our gene-agnostic approach. Unlike traditional gene therapies and gene editing, Ocugen's modifier gene therapies address the entire disease—complex diseases that are potentially caused by imbalances in multiple gene networks. Currently we have programs in development for inherited retinal diseases and blindness diseases affecting millions across the globe, including retinitis pigmentosa, Stargardt disease, and geographic atrophy—late stage dry age-related macular degeneration. 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 OCU410ST 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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