Ocugen, Inc. Announces First Patient Dosed in Phase 2/3 GARDian3 Pivotal Confirmatory Trial for OCU410ST—Novel Modifier Gene Therapy Candidate for Stargardt Disease

July 18, 2025

MALVERN, Pa., July 18, 2025 (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 has been dosed in its Phase 2/3 GARDian3 clinical trial for OCU410ST (AAV5-*hRORA*)—a modifier gene therapy candidate being developed for all Stargardt disease (*ABCA4*-associated retinopathies).

“Dosing the first patient is an especially significant milestone and brings us closer to our goal of addressing the unmet medical need that exists for all Stargardt patients—100,000 in the U.S. and Europe and 1 million worldwide,” said Dr. Shankar Musunuri, Chairman, CEO, and Co-founder of Ocugen. “Progressing our second modifier gene therapy candidate into a registration clinical trial is a pivotal step in potentially providing a one-time therapy for life for the millions of patients affected by inherited retinal diseases.”

The Phase 2/3 clinical trial for OCU410ST builds upon encouraging results and positive data from the Phase 1 GARDian trial, which demonstrated 48% slower lesion growth at 12-month follow up in evaluable treated eyes compared to untreated eyes. Additionally, evaluable treated eyes showed a statistically significant (p=0.031) and clinically meaningful improvement of nearly 2-line gain in best corrected visual acuity (BCVA) at 12-month follow-up when compared to untreated eyes.

“Initiating dosing in this pivotal Phase 2/3 study is an important advancement for Ocugen and more importantly for the Stargardt community," said Dr. Huma Qamar, Chief Medical Officer of Ocugen. “The adaptive design of this trial, including a masked interim analysis at 8 months on 24 subjects, enables us to efficiently evaluate early signals of efficacy and safety while optimizing study conduct. This ensures we generate robust and meaningful data to support our regulatory submissions for approvals.”

"Treating the first patient with this novel gene therapy in the GARDian3 trial is a proud and hopeful moment for our team and for families affected by Stargardt disease," said Victor H. Gonzalez, MD, Principal Investigator and retinal surgeon at Valley Retina Institute, McAllen, Texas. "For decades, patients have faced the progressive loss of central vision with no approved treatment options. The encouraging Phase 1 results give us confidence that OCU410ST could meaningfully slow disease progression and help preserve vision. This trial brings us closer to the possibility of a one-time gene therapy that could transform patients’ quality of life for years to come."

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 Phase 2/3 study will enroll 51 participants diagnosed with Stargardt disease. Of these, 34 will receive a one-time subretinal injection of OCU410ST (200 µL at a concentration of 1.5×10^{11} vector genomes/mL) in the eye with poorer visual acuity, while 17 will be assigned to an untreated control group. The primary objective of the trial is to evaluate the reduction in atrophic lesion size. Key secondary endpoints include improvements in BCVA and low luminance visual acuity (LLVA), compared to controls. Data from the one-year follow-up will be used to support the company’s planned Biologics License Application (BLA).

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

About OCU410ST
OCU410ST utilizes an AAV delivery platform for the retinal delivery of the *RORA* (RAR-Related Orphan Receptor A) gene. It represents Ocugen’s modifier gene therapy approach, which is based on Nuclear Hormone Receptor (NHR) RORA that regulates pathophysiological pathways linked to Stargardt disease, such as lipofuscin formation, oxidative stress, complement formation, inflammation, and cell survival networks.

About Stargardt Disease
Stargardt disease is a genetic eye disorder that causes retinal degeneration and vision loss. Stargardt disease is the most common form of inherited macular degeneration. The progressive vision loss associated with Stargardt disease is caused by the degeneration of photoreceptor cells in the central portion of the retina called the macula.

Decreased central vision due to loss of photoreceptors in the macula is the hallmark of Stargardt disease. Some peripheral vision is usually preserved. Stargardt disease typically develops during childhood or adolescence, but the age of onset and rate of progression can vary. The retinal pigment epithelium (RPE), a layer of cells supporting photoreceptors, is also affected in people with Stargardt disease.

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 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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