Ocugen Announces Publication of Phase 1 GARDian1 Trial Results for OCU410ST Modifier Gene Therapy

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- *Study supports favorable safety and tolerability profile and clinically meaningful functional and structural benefits in Stargardt disease patients*

MALVERN, Pa., Jan. 12, 2026 (GLOBE NEWSWIRE) -- Ocugen, Inc. (Ocugen or the Company) (NASDAQ: OCGN), a pioneering biotechnology leader in gene therapies for blindness diseases, today announced the publication of positive Phase 1 GARDian1 trial results for OCU410ST, its novel modifier gene therapy for Stargardt disease, in the peer-reviewed journal *Nature Eye* —published by the Royal College of Ophthalmologists.

The publication, *A novel modifier gene therapy to treat Stargardt disease: Phase 1 GARDian1 Trial Insights*, was authored by Arshad M. Khanani, MD, MA, FASRS, Director of Clinical Research, and Director of Fellowship at Sierra Eye Associates and Clinical Professor at the University of Nevada, Reno School of Medicine; Lejla Vajzovic, MD, FASRS, Professor of Ophthalmology, Director of CME-Ophthalmology, Duke University School of Medicine; Benjamin A. Bakall, MD, PhD. Associated Retina Consultants, President and Founder, Retina Research Foundation of America, Clinical Assistant Professor, University of Arizona College of Medicine; and Ocugen researchers Dr. Murthy Chavali and Dr. Huma Qamar. The publication reports comprehensive 12-month safety, tolerability, and exploratory efficacy data from the first-in-human Phase 1 trial evaluating OCU410ST in patients with early to advanced Stargardt disease.

The Phase 1 GARDian1 trial demonstrated robust efficacy and safety outcomes supporting the clinical development of OCU410ST:

Key Findings include:

- Among six patients with gradable Fundus Auto Fluorescence images, atrophic lesion growth was reduced by 54% (0.55 ± 0.27 mm²) in treated eyes, compared to untreated fellow eyes (1.19 ± 0.31 mm²) over 12 months
- Lesion expansion was 50% slower (0.10 ± 0.039 mm/year) in treated eyes versus untreated eyes (0.19 ± 0.026 mm/year), below published natural history rates (0.14–0.18 mm/year)
- Among six BCVA-evaluable patients without confounders, treated eyes gained +6 letters in BCVA (+4.5 letters) compared to a −1.5 letter decline in untreated fellow eyes at 12 months
- 100% of treated eyes either stabilized (±4 letters) or improved (≥5 letters) in visual acuity
- No drug-related serious adverse events or adverse events of special interest were observed

Stargardt disease is the most common form of inherited macular degeneration, affecting more than 100,000 people in the United States and Europe combined. The disease is characterized by progressive central vision loss due to photoreceptor degeneration caused by toxic lipofuscin accumulation in the retinal pigment epithelium. Currently, no approved treatment exists for this devastating condition, representing a critical unmet medical need.

"This publication in *Eye* validates the scientific approach and clinical promise of OCU410ST as a modifier gene therapy for Stargardt disease," said Dr. Huma Qamar, Chief Medical Officer at Ocugen. "The Phase 1 GARDian1 trial demonstrated convergent functional and structural benefits. This represents a paradigm shift from any other approaches, including oral or mutation-constrained replacement approaches, to an agnostic modification strategy that can potentially benefit patients regardless of their underlying *ABCA4* mutation with a potential single gene therapy for life. These results provide important support for our ongoing Phase 2/3 GARDian3 trial."

"The consistent benefits observed across both structural and functional endpoints including slowing atrophic lesion progression and stabilization or improvement in visual acuity highlight the potential of this modifier gene therapy platform approach to transform treatment outcomes for patients with Stargardt disease, who currently have no disease-modifying options available," said Dr. Arshad M. Khanani, lead author of the publication, Director of Clinical Research at Sierra Eye Associates, and Ocugen Scientific Advisory Board member. "I am looking forward to the data read out from the ongoing Phase 2/3 GARDian3 trial."

The Phase 2/3 GARDian3 trial is progressing ahead of schedule with anticipated enrollment completion in the first quarter of 2026. The Company remains positioned for Biologics License Application (BLA) filing in the first half of 2027, aligned with its strategy to advance three regulatory submissions in three years.

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.

About Stargardt Disease
Stargardt disease type 1 is a genetic eye disorder caused by biallelic mutations in the *ABCA4* gene. The 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 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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