Ocugen, Inc. Announces U.S. FDA Clearance of Investigational New Drug Amendment to Initiate Phase 2/3 Pivotal Confirmatory Clinical Trial of OCU410ST—Modifier Gene Therapy Candidate for Stargardt Disease

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MALVERN, Pa., June 16, 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 U.S. Food and Drug Administration (FDA) has cleared the Investigational New Drug (IND) amendment to initiate a Phase 2/3 pivotal confirmatory trial of OCU410ST, a modifier gene therapy candidate being developed for all Stargardt disease (*ABCA4*-associated retinopathies). The FDA previously granted Rare Pediatric Disease Designation (RPDD) and Orphan Drug Designation for OCU410ST for the treatment of *ABCA4*-associated retinopathies including Stargardt disease, retinitis pigmentosa 19, and cone-rod dystrophy 3.

"We have had a highly productive and collaborative engagement with the FDA's Center for Biologics Evaluation and Research (CBER) in establishing the pivotal confirmatory trial for OCU410ST," said Dr. Shankar Musunuri, Chairman, CEO and Co-Founder of Ocugen. "It's evident that there is a real sense of urgency by the agency in providing treatment options for patients who currently have nothing available to them. As we initiate the Phase 2/3 registration trial, we are expediting the clinical development of OCU410ST by two to three years and potentially providing an innovative gene therapy to patients desperate for a treatment option."

Positive data from the Phase 1 GARDian trial for OCU410ST demonstrated:

- A favorable safety and tolerability profile with no serious adverse events related to OCU410ST, including no cases of ischemic optic neuropathy, vasculitis, intraocular inflammation, endophthalmitis or choroidal neovascularization and no adverse events of special interest
- Considerably slower lesion growth—48% at 12-month follow up in evaluable treated eyes when compared to untreated eyes
- Statistically significant (p=0.031) improvement with clinically meaningful, nearly 2-line gain in visual function (BCVA) at 12-month follow-up in evaluable treated eyes when compared to untreated eyes

The Phase 2/3 clinical trial for OCU410ST 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 best corrected visual acuity (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 Biologics License Application (BLA).

"The initiation of this pivotal Phase 2/3 study represents a significant milestone in our commitment to bringing transformative genetic therapies to individuals affected by Stargardt disease—a progressive and debilitating condition," said Dr. Huma Qamar, Chief Medical Officer at Ocugen. "The recent RPDD granted by the FDA for this program further underscores the urgent need for innovative treatment options for children living with Stargardt disease. OCU410ST, developed through our proprietary modifier gene therapy platform, is designed to target the underlying biological mechanisms of the disease."

Approximately 100,000 patients in U.S. and Europe combined and 1 million patients globally live with Stargardt disease. Stargardt and *ABCA4*-associated retinopathies are genetically complex, involving more than 1,200 known mutations and addressing this condition with traditional gene therapy or gene editing approaches remains highly challenging.

"Stargardt disease represents a significant unmet medical need, particularly among children and young adults," said Lejla Vajzovic, MD, FASRS, Director of the Duke Surgical Vitreoretinal Fellowship Program and Professor of Ophthalmology, Pediatrics, and Biomedical Engineering with Tenure at Duke University Eye Center. "The Phase 2/3 study of OCU410ST is thoughtfully designed with scientific rigor and a patient-centered focus to evaluate both structural and functional outcomes. We are optimistic that this approach will move us closer to a meaningful therapeutic solution for affected families."

The OCU410ST Phase 2/3 pivotal confirmatory trial represents a major advancement as 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.

Contact:
Tiffany Hamilton
AVP, Head of Communications
Tiffany.Hamilton@ocugen.com