Ocugen, Inc. Announces FDA Alignment on Phase 2/3 Pivotal Confirmatory Clinical Trial for Modifier Gene Therapy Candidate OCU410ST for Stargardt Disease

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MALVERN, Pa., Feb. 27, 2025 (GLOBE NEWSWIRE) -- Ocugen, Inc. (Ocugen or the Company) (NASDAQ: OCGN), a pioneering biotechnology leader in gene therapies for blindness diseases, today announced that alignment has been reached with the U.S. Food and Drug Administration (FDA) to move forward with a Phase 2/3 pivotal confirmatory clinical trial for OCU410ST which, if positive, can be the basis of a biologics license application (BLA) submission.

The 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 (52%) from baseline in treated eyes versus untreated fellow eyes at 6-month follow-up from the Phase 1 study
- Clinically meaningful 2-line (10-letter) improvement in visual function (BCVA) at 6-month follow-up from the Phase 1 study, which is statistically significant (p=0.02) in treated eyes

“I am very pleased that the FDA has recognized the promise of Ocugen’s modifier gene therapy for Stargardt disease and accelerated the regulatory pathway for OCU410ST,” said Dr. Shankar Musunuri, Chairman, CEO, and Co-founder of Ocugen. “This new development allows us to initiate a pivotal confirmatory trial for this game-changing, one-time treatment for life in the next few months and prepare for a potential BLA filing by 2027. Now patients suffering from Stargardt disease have a new hope where previously none existed. This achievement furthers our mission to cure blindness diseases.”

Stargardt disease affects 100,000 people in the U.S. and Europe combined, and there is no treatment. OCU410ST received orphan drug designations from the FDA and the European Medicines Agency (EMA) in 2023 and 2024, respectively.

“Getting approval for a Phase 2/3 trial is a pivotal milestone, as this approach has never been explored in clinical trials for Stargardt disease. The FDA’s decision underscores the potential of OCU410ST to meet a critical unmet medical need for the approximately 44,000 Stargardt patients in the U.S.,” said Lejla Vajzovic, MD, FASRS, Director, Duke Surgical Vitreoretinal Fellowship Program, Professor of Ophthalmology, Pediatrics and Biomedical Engineering with Tenure, Adult and Pediatric Vitreoretinal Surgery and Disease, Duke University Eye Center, and Retina Scientific Advisory Board Chair of Ocugen.

The Phase 2/3 clinical trial will randomize 51 subjects, 34 of whom will receive a single, subretinal, 200-µL injection of OCU410ST at a concentration of 1.5×10^{11} vector genomes (vg)/mL in the eye with worse visual acuity, and 17 of whom will serve as untreated controls. The primary endpoint in the clinical trial is change in atrophic lesion size. Secondary endpoints include visual acuity as measured by best corrected visual acuity (BCVA) and low luminance visual acuity (LLVA) compared to untreated controls. One-year data will be utilized for the BLA filing.

“This approval pathway, established in collaboration with the FDA, has made it possible to expedite the clinical development of OCU410ST by two to three years and has aided in bringing an innovative gene therapy to patients desperate for a treatment option,” said Dr. Huma Qamar, Chief Medical Officer at Ocugen. “Recent data from the OCU410ST clinical trial have shown significant improvements in both structural and functional outcomes. Additionally, OCU410ST has consistently demonstrated a very favorable safety and tolerability profile.”

Accelerating the clinical timeline of OCU410ST will save significant costs in addressing disease burden even sooner than anticipated.

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 and cell therapies, biologics, and vaccines that improve health 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 treat multiple retinal diseases with a single product, and we are advancing research in infectious diseases to support public health and orthopedic diseases to address unmet medical needs. 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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