Ocugen Announces Data and Safety Monitoring Board Approves Enrollment in High Dose Cohort 3 in GARDian Study for Stargardt Disease

June 21, 2024

- *Established Medium Dose as Safe and Tolerable Dose in Current OCU410ST Clinical Trial*
- *DSMB Determination to Proceed with High Dose Cohort Dosing*

MALVERN, Pa., June 21, 2024 (GLOBE NEWSWIRE) -- Ocugen, Inc. (Ocugen or the Company) (NASDAQ: OCGN), a biotechnology company focused on discovering, developing, and commercializing novel gene and cell therapies and vaccines, today announced that the Data and Safety Monitoring Board (DSMB) for the OCU410ST GARDian clinical trial recently convened and approved to proceed with dosing the high dose of OCU410ST in the dose-escalation phase of the study. OCU410ST (*AAV5-hRORA*) is a modifier gene therapy candidate being developed for Stargardt disease. Stargardt disease affects approximately 100,000 people in the U.S. and Europe combined.

Six patients with Stargardt disease have been dosed in the Phase 1/2 clinical trial to date in the low dose cohort and medium dose cohort. An additional three patients will be dosed with the high dose in cohort 3.

“The DSMB has recommended moving forward to dose subsequent subjects with Stargardt disease at the targeted high dose,” said Dr. Peter Y. Chang, MD, FACS, DSMB Chair for the OCU410ST clinical trial. “No serious adverse events (SAEs) related to OCU410ST have been reported to date. This is an important next step in the clinical progress for OCU410ST and encouraging for patients living with this most common form of inherited retinal disease.”

“We are delighted to report a second positive DSMB recommendation for the treatment of Stargardt disease and build upon the favorable safety and tolerability profile exhibited by OCU410ST,” said Huma Qamar, M.D., MPH, Chief Medical Officer of Ocugen. "We recognize the high unmet medical need for Stargardt patients as there is no approved product. We are enthusiastic about OCU410ST as a potential one-time treatment for life with a single sub-retinal injection. We look forward to sharing a clinical trial update later this year."

The Phase 1/2 GARDian clinical trial will include up to 42 subjects—30 adults and 12 children with Stargardt disease—who exhibit mild to moderate disease symptoms and will assess the safety of unilateral subretinal administration of OCU410ST. The clinical trial is being conducted in two phases. Phase 1 is a multicenter, open-label, dose-ranging/dose-escalation study consisting of three dose levels [low dose (3.75×10^{10} vg/mL), medium dose (7.5×10^{10} vg/mL), and high dose (2.25×10^{11} vg/mL)]. Phase 2 is a randomized, outcome accessor-blinded, dose-expansion study in which adult and pediatric subjects will be enrolled in a 1:1:1 ratio to randomize subjects into two different treatment groups at varying dose levels, or a control (untreated group), allowing for a comprehensive assessment of the treatment's efficacy across different dosages.

Currently, patients with Stargardt disease have no FDA-approved therapeutic options. Ocugen is dedicated to providing a gene-agnostic treatment approach for patients living with inherited retinal diseases and is encouraged that the Phase 1/2 GARDian trial for OCU410ST remains on track.

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 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 pathway links to Stargardt disease such as lipofuscin formation, oxidative stress, complement formation, inflammation, and cell survival networks.

About Ocugen, Inc.
Ocugen, Inc. is a biotechnology company focused on discovering, developing, and commercializing novel gene and cell therapies 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; 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
Head of Communications
Tiffany.Hamilton@ocugen.com