Ocugen Announces Dosing Completion of Subjects with Geographic Atrophy in Cohort 2 of Phase 1/2 ArMaDa Clinical Trial of OCU410—A Modifier Gene Therapy

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MALVERN, Pa., April 19, 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 dosing is complete in the second cohort of its Phase 1/2 ArMaDa clinical trial for OCU410 (AAV-*hRORA*)—a modifier gene therapy candidate being developed for geographic atrophy (GA), an advanced stage of dry age-related macular degeneration (dAMD). GA affects approximately 1 million people in the United States alone.

“We are very enthusiastic about the potential of OCU410 as a one-time, gene-agnostic option for the treatment of GA,” said Dr. Huma Qamar, Chief Medical Officer of Ocugen. “OCU410 regulates multiple pathways involved with the disease, including lipid metabolism, inflammation, oxidative stress, and membrane attack complex (complement) with a single sub-retinal injection.”

Dosing in the second cohort is complete and 3 subjects received 200 mL single subretinal administration of the medium dose ($5x10^{10}$ vg/mL) of OCU410. Up to 13 leading retinal surgery centers across the United States are participating in the ArMaDa clinical trial.

“Currently we have two FDA approved, anti-complement therapies for GA targeting a single pathway of the disease, which has multifactorial and complex etiology,” said Syed M. Shah, MD, Vice Chair of Research and Digital Medicine and Director of Retina Service at Gundersen Health System, La Crosse, WI, and the lead investigator for the OCU410 Phase 1/2 trial. “The limited benefit comes with the burden of continued multiple intravitreal injections spanning over several years. This novel modifier gene therapy has the potential to transform the therapeutic landscape in GA treatment.”

A Data and Safety Monitoring Board meeting will convene next month to review the 4-week safety data of the medium dose cohort before proceeding with high dose, which is the final dose in the Phase 1 dose-escalation study.

The ArMaDa Phase 1/2 clinical trial will assess the safety of unilateral subretinal administration of OCU410 in subjects with GA and will be conducted in two phases. Phase 1 is a multicenter, open-label, dose-ranging study consisting of three dose levels [low dose (2.5×10^{10} vg/mL), medium dose (5×10^{10} vg/mL), and high dose (1.5×10^{11} vg/mL). Phase 2 is a randomized, outcome accessor-blinded, dose-expansion study in which subjects will be randomized in a 1:1:1 ratio to either one of two OCU410 treatment groups or to an untreated control group.

The Company will continue to provide clinical updates on an ongoing basis.

About dAMD and GA
dAMD affects approximately 10 million Americans and more than 266 million people worldwide. It is characterized by the thinning of the macula. The macula is the part of the retina responsible for clear vision in one’s direct line of sight. dAMD involves the slow deterioration of the retina with submacular drusen (small white or yellow dots on the retina), atrophy, loss of macular function and central vision impairment. dAMD accounts for 85-90% of the total AMD population.

About OCU410
OCU410 utilizes an AAV delivery platform for the retinal delivery of the *RORA* (ROR Related Orphan Receptor A) gene. The RORA protein plays an important role in lipid metabolism, reducing lipofuscin deposits and oxidative stress, and demonstrates an anti-inflammatory role as well as inhibiting the complement system in in-vitro and in-vivo (animal model) studies. These results demonstrate the ability for OCU410 to target multiple pathways linked with dAMD pathophysiology. Ocugen is developing *AAV-RORA* as a one-time gene therapy for the treatment of GA.

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 patients’ 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.

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.

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