Ocugen, Inc. Announces First Patient Dosed in Phase 1/2 Clinical Trial Evaluating the Safety and Efficacy of OCU410—Modifier Gene Therapy—for Geographic Atrophy Secondary to Dry Age-Related Macular Degeneration

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MALVERN, Pa., Dec. 13, 2023 (GLOBE NEWSWIRE) -- Ocugen, Inc. (“Ocugen” or the “Company”) (NASDAQ: OCGN), a biotechnology company focused on discovering, developing, and commercializing novel gene and cell therapies, biologics, and vaccines, today announced that the first patient has been dosed in the ArMaDa Phase 1/2 clinical trial of OCU410 (AAV-*RORA),* a modifier gene therapy product candidate being developed for dry AMD (dAMD).

“OCU410, our first-in-class modifier gene therapy for dAMD, addresses gaps among other therapies available and in development for dAMD as a potential one-time treatment for life,” said Dr. Shankar Musunuri, Chairman, CEO and Co-Founder of Ocugen. “We are very pleased to continue advancing our ophthalmic gene therapy pipeline, which remains the Company’s primary focus.”

This Phase 1/2 trial will assess the safety and efficacy of OCU410 for geographic atrophy (GA) secondary to dAMD and will be conducted in two phases. Phase 1 is a multicenter, open-label, dose-ranging study. Phase 2 is a randomized expansion phase in which subjects will be randomized in a 1:1:1 ratio to either one of two OCU410 dose groups or to an untreated control group.

OCU410 is a potential curative therapy with a single sub-retinal injection that targets multiple pathways causing dAMD, including lipid metabolism, inflammation, oxidative stress, and complement activation. Currently, the other therapeutic options available target only complement activation and require approximately 6-12 intravitreal injections annually.

“Breaking new ground in the pursuit of vision restoration, our pioneering modifier gene therapy candidate, OCU410, achieves another major milestone by dosing a GA patient in a Phase 1/2 clinical trial,” said Arun Upadhyay, PhD, Chief Scientific Officer, Head of R&D at Ocugen. “OCU410 offers hope for those battling GA that are faced with limited treatment options and the real prospect of ultimately losing their vision.”

“There remains a great unmet need for novel durable and effective treatments for GA, which remains one of the most common causes of vision loss globally,” said Benjamin Bakall, MD, PhD, director of clinical research at Associated Retina Consultants (ARC) and clinical assistant professor at University of Arizona, College of Medicine – Phoenix. “I am excited that we performed the first surgery with this novel therapeutic approach—designed to restore homeostasis and slow disease progression following a single treatment—at ARC in Phoenix, AZ, with the surgical team led by Dr. Mark Kwong, medical director of ARC.”

The first surgery was successful in delivering the new gene underneath the retina; the light sensitive nerve tissue lining the inside of the eye.

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.

GA, an advanced form of dry age-related macular degeneration, affects approximately 1 million people in the United States alone.

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 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. Currently, the other therapeutic options available target only complement activation and require approximately 6-12 intravitreal injections annually.

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.

About Associated Retina Consultants
Associated Retina Consultants is the largest independent retina practice in the state of Arizona, taking part in groundbreaking clinical trials to bring new treatments for the benefit of patients with diseases affecting the retina. In addition to collaborating with Ocugen on the OCU410 clinical trial, in October 2023, Associated Retina Consultants—with a surgical team led by Dr. Mark Kwong—performed gene therapy with OCU400 on a pediatric *CEP290* patient. This was the first retinal gene therapy of its kind performed on a child in Arizona.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995, 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. 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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