Ocugen, Inc. Announces Dosing Completion of Subjects with Geographic Atrophy in Cohort 1 of Phase 1/2 Clinical Trial Evaluating the Safety and Efficacy of OCU410

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MALVERN, Pa., March 13, 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 first 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 treatment for life with a single sub-retinal injection,” said Dr. Shankar Musunuri, Chairman, CEO and Co-Founder of Ocugen. “While there are currently two recently approved products for the treatment of GA, both require approximately 6-12 intravitreal injections annually and target only the complement system. OCU410 addresses multiple pathways causing dAMD, including complement, lipid metabolism, inflammation, and oxidative stress.”

Up to 13 leading retinal surgery centers across the United States are participating in the ArMaDa clinical trial. The enrollment in the first cohort is now complete and 3 subjects received 200µL single subretinal administration of the low dose ($2.5x10^{10}$ vg/mL) of OCU410.

“As a retinal surgeon, I am encouraged by therapeutic options that can potentially provide long-term benefit to my patients,” said Lejla Vajzovic, MD, FASRS, Director of Duke Surgical Vitreoretinal Fellowship Program, Associate Professor of Ophthalmology with Tenure in Adult and Pediatric Vitreoretinal Surgery and Diseases, Duke University Eye Center. “OCU410 is a novel modifier gene therapy approach that could initiate a paradigm shift in the field of ophthalmology.”

The ArMaDa 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 American Macular Degeneration Foundation (AMDF) has supported the research of Dr. Neena Haider, inventor of modifier gene therapy, and OCU410 in particular, and is pleased that Ocugen is now spearheading the clinical trials necessary to bring this therapy closer to patients,” said Matthew Levine, Director of Grants, Advocacy and Partnerships at AMDF. “The continued advancement of OCU410 offers hope to those whose vision is already deteriorating that their remaining vision could be preserved and could potentially prevent others with an early dAMD diagnosis from developing any significant vision loss.”

The Company will continue to provide clinical updates.

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 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, 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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