



Ocugen, Inc. Announces First Patient Dosed in Phase 1/2 Clinical Trial Evaluating the Safety and Efficacy of OCU410ST—Modifier Gene Therapy—for Stargardt Disease

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MALVERN, Pa., Nov. 10, 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 its Phase 1/2 GARDian clinical trial for OCU410ST (AAV5-*hRORA*)—a modifier gene therapy candidate being developed for Stargardt disease, a rare genetically inherited disease that directly affects the retina, often resulting in slow progressive vision loss in children and adults.

"There is a significant unmet medical need for the approximate 35,000 patients in the U.S. living with Stargardt disease," said Dr. Shankar Musunuri, Chairman, CEO and Co-Founder of Ocugen. "It is critical to our mission to develop innovative treatments for inherited retinal diseases and this milestone is an important step in bringing our novel modifier gene therapies to people who desperately need them."

This Phase 1/2 trial will assess the safety of unilateral subretinal administration of OCU410ST in subjects with Stargardt Disease and will be conducted in two phases. Phase 1 is a multicenter, open-label, dose ranging study. Phase 2 is a randomized, outcome assessor-blinded, dose-expansion study in which adult and pediatric subjects will be randomized in a 1:1:1 ratio to either one of two OCU410ST dose groups or to an untreated control group.

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.

"It is important and exciting to pursue novel therapies for untreatable blinding diseases," said Charles Wykoff, MD, PhD, Director of Research, Retina Consultants of Texas. "Initiation of this trial program investigating a new mechanism of action for the treatment of Stargardt disease is inspiring and brings hope to patients and families."

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 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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