Ocugen, Inc. Announces First Patient Dosed in Phase 1/2 Clinical Trial for Gene Therapy Candidate OCU400 to Treat Inherited Retinal Degeneration

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Announcement marks first clinical trial in humans of Ocugen's modifier gene therapy platform

MALVERN, Pa., April 01, 2022 (GLOBE NEWSWIRE) -- Ocugen, Inc. (NASDAQ: OCGN), a biotechnology company focused on discovering, developing, and commercializing novel gene therapies, biologicals and vaccines, announced that the first patient has been dosed in the Phase 1/2 clinical trial of OCU400, a modifier gene therapy candidate for the treatment of retinitis pigmentosa (RP) resulting from mutations in the nuclear receptor subfamily 2 group E member 3 *(NR2E3)* and Rhodopsin (*RHO*) genes.

This first patient dosing marks the beginning of the dose-escalating, observer-blind, Phase 1/2 safety and efficacy study. This Phase 1/2 study is currently enrolling. More information can be found on ClinicalTrials.gov, under identifier number NCT05203939.

"Everyone at Ocugen is excited about this important milestone. Every day, our teams are working toward developing a therapeutic for people who have no options when facing inherited retinal diseases," said Dr. Shankar Musunuri, Chairman of the Board, Chief Executive Officer, and Co-Founder of Ocugen. "The first phase of the study is a safety evaluation of the product, eventually progressing into an efficacy study in patients. Today's announcement signifies a first and monumentally critical step forward in achieving our mission to cure blindness diseases."

RP is a group of rare, genetic disorders that involve a breakdown and loss of cells in the retina (the light-sensitive tissue that lines the back of the eye). Common symptoms include difficulty seeing at night and a progressive loss of side (peripheral) vision. It is generally estimated that RP affects roughly 1 in 4,000 people – approximately two million people – globally.[1] There is currently no approved therapy intended to stop the progression of RP based on all of the genetic mutations that cause the disease.

Ocugen's modifier gene therapy platform aims to target nuclear hormone receptors (NHRs) that regulate multiple functions within the retina, giving it the potential to address many different gene mutations—and, in turn, multiple retinal diseases—with a single product. Traditional gene therapy, which transfers a functional version of a non-functional gene into target cells, addresses only one individual gene mutation at a time.

"Our premise is that disease progression can be halted at whatever stage patients are currently at, potentially preventing further vision loss," said David Birch, PhD, Scientific Director at the Rose-Silverthorne Retinal Degenerations Laboratory. "This Phase 1/2 clinical trial targets people who have RP resulting from mutations in the *NR2E3 and RHO* genes. Based on the safety and efficacy outcomes, this study may be expanded to include additional genetic mutations in a Phase 3 study designed to demonstrate broad therapeutic applications of OCU400 in people with RP and Leber congenital amaurosis. If approved, we believe OCU400 may ultimately impact the lives of people facing retinitis pigmentosa and other retinal diseases rooted in the mutations of more than 175 genes."

About Ocugen, Inc.
Ocugen, Inc. is a biotechnology company focused on discovering, developing, and commercializing novel gene therapies, biologicals and vaccines that improve health and offer hope for people and global communities. We are making an impact through courageous innovation, taking science in new directions in service of patients. Our breakthrough modifier gene therapy platform has the potential to treat multiple diseases with one drug and we are advancing research in other therapeutic areas to offer new options for people with unmet medical needs. Discover more at www.ocugen.com and follow us on Twitter 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, 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 forward-looking statements include information about qualitative assessments of available data, potential benefits, expectations for clinical trials, and anticipated timing of clinical trial readouts and regulatory submissions, including with respect to our planned Phase 1/2 trial included in our Investigational New Drug application to the U.S. Food and Drug Administration (FDA) for OCU400, which was recently accepted by the FDA. This information involves risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statements. Risks and uncertainties include, among other things, the uncertainties inherent in research and development, including the ability to meet anticipated clinical endpoints, commencement and/or completion dates for clinical trials, regulatory submission dates, regulatory approval dates and/or launch dates, as well as risks associated with preliminary and interim data, including the possibility of unfavorable new clinical trial data and further analyses of existing clinical trial data; the risk that the results of in-vitro studies will not be duplicated in human clinical trials; the risk that clinical trial data are subject to differing interpretations and assessments, including during the peer review/publication process, in the scientific community generally, and by regulatory authorities; and the risk that the Orphan Drug Designations from the FDA and broad Orphan Medicinal Product Designation from the European Commission for OCU400 may not result in a faster approval timeline for OCU400 or increase the likelihood of any such approvals 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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References:

1. Retinitis pigmentosa. National Organization of Rare Disorders. https://rarediseases.org/rare-diseases/retinitis-pigmentosa/ (Accessed March 28, 2022)