Ocugen, Inc. Announces U.S. FDA Acceptance of Investigational New Drug Application to Initiate a Phase 1/2 Clinical Trial for Gene Therapy Candidate OCU400 to Treat Inherited Retinal Degeneration

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- *Gene therapy candidate has potential to address a large number of retinitis pigmentosa and Leber congenital amaurosis gene mutations with a single product*
- *Trial to start in Q1 2022 will enroll patients with mutations in NR2E3 or RHO genes*

MALVERN, Pa., Dec. 09, 2021 (GLOBE NEWSWIRE) -- Ocugen, Inc. (NASDAQ: OCGN), a biopharmaceutical company focused on discovering, developing, and commercializing gene therapies to cure blindness diseases and developing a vaccine to fight COVID-19, announced that the U.S. Food and Drug Administration (FDA) has accepted the company's Investigational New Drug application (IND) to initiate a first-in-human clinical trial of OCU400 (*AAV-NR2E3*), a modifier gene therapy candidate for the treatment of retinitis pigmentosa resulting from genetic mutations found in NR2E3 and Rhodopsin.

"We are delighted to advance OCU400 into clinical trials, which exemplifies our goal of offering new options to people with genetic diseases where none currently exist," said Shankar Musunuri, PhD, MBA, Chairman of the Board, Chief Executive Officer, and Co-Founder of Ocugen. "We're collaborating with leading centers in eye care and have been vital partners to getting our trial launched and receive patients. With this final decision by the FDA, we are embarking on a new pathway of care through this innovative gene therapy."

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, targets only one individual gene mutation at a time.

OCU400 was granted four orphan drug disease designations from the FDA for treating four different gene mutation-associated retinal degenerative diseases between 2019 and 2020. The European Medicines Agency (EMA) granted Ocugen broad orphan medicinal product designation in 2021 for OCU400 for the treatment of both retinitis pigmentosa (RP) and Leber congenital amaurosis (LCA) – meaning that, if approved, OCU400 by itself could treat these diseases that are rooted in mutations of more than 175 different genes.

"Ocugen's game-changing approach to gene therapy could provide mutation agnostic therapies that raise the bar on how we could treat genetic diseases in the future," said Mark Pennesi, MD, PhD, Professor of Ophthalmology and Chief of the Paul H. Casey Ophthalmic Genetics Division, Oregon Health & Science University, and member of Ocugen's Retina Scientific Advisory Board.

Details on this clinical trial will be available in the coming weeks on www.clinicaltrials.gov.

About Ocugen, Inc.
Ocugen, Inc. is a biopharmaceutical company focused on discovering, developing, and commercializing gene therapies to cure blindness diseases and developing a vaccine to save lives from COVID-19. Our breakthrough modifier gene therapy platform has the potential to treat multiple retinal diseases with one drug – "one to many" and our novel biologic product candidate aims to offer better therapy to patients with underserved diseases such as diabetic macular edema, wet age-related macular degeneration, and diabetic retinopathy. We are co-developing Bharat Biotech's COVAXIN™ vaccine candidate for COVID-19 in the U.S. and Canadian markets. For more information, please visit www.ocugen.com.

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 (IND) 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; the risk that the orphan drug designations from the FDA and broad orphan medicinal product designation from the European Medicines Agency for OCU400 may not result in a faster approval timeline for OCU400 or increase the likelihood of any such approvals; whether developments with respect to the COVID-19 pandemic will affect the regulatory pathway available for vaccines in the United States, Canada or other jurisdictions; market demand for COVAXIN™ in the United States or Canada; decisions by the FDA or Health Canada impacting labeling, manufacturing processes, safety and/or other matters that could affect the availability or commercial potential of COVAXIN™ in the United States or Canada, including development of products or therapies by other companies. 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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