Ocugen, Inc. Announces Submission of Investigational New Drug Application with U.S. FDA to Initiate a Phase 1/2 Clinical Trial Evaluating Gene Therapy Candidate OCU400 (AAV-NR2E3) to Treat Inherited Retinal Degeneration

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Modifier gene therapy platform has potential to treat multiple retinal diseases with one product

MALVERN, Pa., Nov. 08, 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 it has submitted an Investigational New Drug application (IND) with the U.S. Food and Drug Administration (FDA) to initiate a Phase 1/2 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 *RHO*.

"This important milestone brings us one step closer towards achieving our company's vision of fostering a future where no one feels hopeless in the face of disease," said Dr. Shankar Musunuri, Chairman of the Board, Chief Executive Officer, and Co-Founder of Ocugen. "Our novel modifier gene therapy platform is a strong example of our approach to drug development."

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.

"The goal with OCU400 (AAV- *NR2E3)* is to offer people living with genetic vision conditions – people whose vision is slowly deteriorating and who have no current treatment options – a reason to hope," commented Arun Upadhyay, PhD, Vice President and Head of Research & Development at Ocugen. "And we're very pleased to be on this journey with the creator of our modifier gene therapy platform, Dr. Neena Haider from Harvard Medical School. Collaboration is a mother of innovation, and our work with Dr. Haider is really driving us to see a whole new potential for care."

The planned Phase 1/2 clinical study will evaluate the safety and proof-of-concept of OCU400, utilizing unilateral sub-retinal injection (one eye per study subject) in 18 patients, with an extension for contralateral eye and long-term safety follow up. Ocugen has already successfully completed manufacturing at commercial scale (200L) to support clinical studies. Ocugen plans to follow the Phase 1/2 trial with a Phase 3 study, upon further review of the data.

Between 2019 and 2020, OCU400 was granted four orphan drug disease designations from the FDA from treating four different gene mutation-associated retinal degenerative diseases. In 2021, the European Medicines Agency (EMA) granted Ocugen broad orphan medicinal product designation 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.

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 wet age-related macular degeneration, diabetic macular edema, 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. 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; whether the FDA will accept our IND submission for OCU400 without any changes, or if we are required to submit additional information to the FDA in support of our IND submission, the extent and significance of any such changes; 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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