



Ocugen, Inc. Announces Positive DSMB Recommendation for OCU400-101 Clinical Trial

April 25, 2022

Phase 1/2 study to assess the safety and efficacy of OCU400 modifier gene therapy candidate to treat Retinitis Pigmentosa associated with NR2E3 and RHO mutations

MALVERN, Pa., April 25, 2022 (GLOBE NEWSWIRE) -- Ocugen, Inc. (NASDAQ: OCGN), a biotechnology company focused on discovering, developing, and commercializing novel gene therapies, biologicals and vaccines, announced today that the independent Data and Safety Monitoring Board (DSMB) for its Phase 1/2 clinical trial of OCU400, the Company's flagship modifier gene therapy candidate for the treatment of Retinitis Pigmentosa (RP), reviewed safety data based on dosing to date and recommended that the study proceed with enrolling additional subjects.

The OCU400-101 clinical study to assess the safety and efficacy of modifier gene therapy candidate OCU400 for RP resulting from mutations in the nuclear receptor subfamily 2 group E member 3 (*NR2E3*) and Rhodopsin (*RHO*) genes recently dosed its first patient. The DSMB recommended that the Company continue enrolling the remaining study subjects in this current cohort at the target dose level.

Ocugen's modifier gene therapy platform targets 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.

"It's a positive first step that the DSMB review of the current OCU400-101 study results identified no serious adverse events and recommended that the study proceed with enrollment," 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. "We're looking forward to understanding how this modifier gene therapy platform could treat inherited retinal degeneration, potentially bringing an option to people affected with this disease."

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 Phase 1/2 trial included in our Investigational New Drug application to the U.S. Food and Drug Administration (FDA) for OCU400, which is actively enrolling patients following review of preliminary safety data by the independent Data and Safety Monitoring Board. 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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