Ocugen Announces Update on OCU400 Phase 1/2 Clinical Trial Targeting Retinitis Pigmentosa and Leber Congenital Amaurosis

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Continued Positive Safety Data for OCU400

Established High Dose as the Maximum Tolerable Dose in Current OCU400 Clinical Trial

MALVERN, Pa., Dec. 07, 2022 (GLOBE NEWSWIRE) -- Ocugen, Inc. (Ocugen or the Company) (NASDAQ: OCGN), a biotechnology company focused on discovering, developing, and commercializing novel gene and cell therapies and vaccines, today announced that the Data Safety and Monitoring Board (DSMB) for the OCU400 clinical trial recently convened and established high dose as the maximum tolerable dose (MTD) in the dose-escalation phase of the study.

"The DSMB has recommended moving forward to dose subsequent subjects with *NR2E3* and *RHO* gene mutations associated with Retinitis Pigmentosa (RP) and *CEP290* gene mutations associated with Leber Congenital Amaurosis (LCA) at the targeted dose in the expansion phase of the study," said Dr. Peter Y. Chang, MD, FACS, Massachusetts Eye Research & Surgery Institution, DSMB Chair for the OCU400 clinical trial. "No serious adverse events (SAEs) related to OCU400 have been reported to date."

10 patients with *NR2E3* and *RHO* gene mutations associated with RP have been dosed in the Phase 1/2 clinical trial to date. An additional eight patients with these RP gene mutations, along with three patients with *CEP290* gene mutations associated with LCA, will be dosed at MTD and enrollment is expected to be complete by the end of Q1 2023.

Data from the MTD has the potential to navigate strategy for the planned Phase 3 study in the U.S. and other major markets. The Company plans to file a Biologics License Application (BLA) for OCU400 in 2025.

Ocugen is committed to finding solutions for people with inherited retinal disease for whom no effective treatment options exist. Currently, RP is associated with mutations in more than 100 genes, affecting more than 2.5 million people globally. LCA is a rare eye disease associated with mutations in more than 25 genes, affecting more than 150,000 people globally.

About Ocugen, Inc.
Ocugen, Inc. is a biotechnology company focused on discovering, developing, and commercializing novel gene and cell therapies and vaccines that improve health and offer hope for patients across the globe. We are making an impact on patient's 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 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 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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