Ocugen Announces FDA Approval for Enrollment of Pediatric Patients in Ongoing OCU400 Phase 1/2 Clinical Trial for the Treatment of Retinitis Pigmentosa (RP) and Leber Congenital Amaurosis (LCA)

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- *U.S. Food & Drug Administration (FDA) approves enrolling pediatric patients in the ongoing OCU400 Phase 1/2 trial who have: 1) RP associated with NR2E3 and RHO mutations and 2) LCA associated with CEP290 gene mutations*

- *Ocugen has completed enrollment of adult RP patients with NR2E3 and RHO mutations in the Phase 1/2 trial and expanded enrollment in LCA patients with CEP290 mutations*

MALVERN, Pa., March 27, 2023 (GLOBE NEWSWIRE) -- Ocugen, Inc. (Ocugen or the Company) (NASDAQ: OCGN), a biotechnology company focused on discovering, developing, and commercializing novel gene and cell therapies, biologics, and vaccines, today announced that the FDA approved enrolling pediatric patients in the ongoing OCU400 Phase 1/2 trial.

“This approval moves us one step closer in our efforts to bring OCU400, a novel gene-agnostic modifier gene therapy, to market as a potential life-changing treatment for children afflicted with inherited retinal diseases, such as RP and LCA,” noted Arun Upadhyay, PhD, Ocugen’s Chief Scientific Officer. “This approval further demonstrates the consistent, positive, and timely progress we are making with the Phase 1/2 trial in adult patients. Since a significant number of individuals in the pediatric age group are diagnosed with RP and LCA, it is very important for us to cover this age group in our clinical trials.”

Enrollment of adult RP patients in the Phase 1/2 trial is complete—per protocol—and enrollment continues among patients with LCA. The Company plans to initiate the Phase 3 trial near the end of 2023.

Unlike single-gene replacement therapies, which only target one genetic mutation, Ocugen believes that its modifier gene therapy platform, through its use of Nuclear Hormone Receptors (NHRs), represents a novel approach that has the potential to address multiple retinal diseases caused by mutations in multiple genes with one product, and potentially address complex diseases that are caused by imbalances in multiple gene-networks. While single-gene replacement therapies have shown tremendous promise in rare retinal diseases, they are highly specific and cannot improve a multitude of disease-causing genetic defects. For example, RP and LCA are associated with mutations in more than 100 and in more than 25 genes, respectively. Ocugen is the only company with a gene-agnostic modifier platform that aims to alter this single-gene therapy paradigm through the introduction of a functional gene to modify the expression of multiple genes and gene-networks. We believe that patient prevalence in the United States alone would provide significant long-term value, with RP and LCA affecting 110,000 and 15,000 people, respectively.

OCU400 is the Company’s gene-agnostic modifier gene therapy product based on NHR gene, *NR2E3*. *NR2E3* regulates diverse physiological functions within the retina—such as photoreceptor development and maintenance, metabolism, phototransduction, inflammation and cell survival networks. Through its diverse functionality, OCU400 resets altered/affected cellular gene-networks and establishes homeostasis—a state of balance, which has the potential to improve retinal health and function in patients with inherited retinal diseases.

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