Ocugen Announces OCU400 Receives Orphan Drug Designations for Retinitis Pigmentosa and Leber Congenital Amaurosis

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U.S. Food & Drug Administration (FDA) acknowledges the potential of OCU400 to treat rare inherited retinal diseases

MALVERN, Pa., Dec. 15, 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 FDA granted orphan drug designations to OCU400—human nuclear hormone receptor subfamily 2 group E member 3 (*NR2E3*)—for the treatment of retinitis pigmentosa (RP) and Leber congenital amaurosis (LCA).

“Receiving orphan drug designation is incredibly encouraging at this stage in the development of OCU400,” said Arun Upadhyay, PhD, Chief Scientific Officer, Ocugen. “We are excited by the potential of OCU400, a nuclear hormone-based modifier gene therapy product, to treat RP and LCA in a gene agnostic manner. We look forward to working collaboratively with the FDA and other agencies to progress OCU400 through clinical development to commercialization.”

Orphan drug designation is a status given to certain drugs that show promise in the treatment, prevention, or diagnosis of orphan diseases. An orphan disease is a rare disease or condition that affects fewer than 200,000 people in the United States.

Currently, RP is associated with mutations in more than 100 genes, affecting approximately 110,000 people in the United States (U.S.). LCA is associated with mutations in more than 25 genes, affecting approximately 10,000 people in the U.S. There are currently no treatment options available for patients living with RP and LCA, and OCU400 has the potential to treat both with a single product.

OCU400 represents Ocugen’s modifier gene therapy approach, which is based on Nuclear Hormone Receptors (NHRs) that regulate diverse physiological functions, such as homeostasis, reproduction, development, and metabolism to potentially improve retinal health and function.

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.

Contact:
Tiffany Hamilton
Head of Communications
IR@ocugen.com