Ocugen Announces OCU410ST Receives Orphan Drug Designation for Treatment of ABCA4-Associated Retinopathies

April 27, 2023

U.S. Food & Drug Administration (FDA) grants orphan drug designation to OCU410ST for the treatment of ABCA4-associated retinopathies including Stargardt, RP19 and CORD3

MALVERN, Pa., April 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 has granted Orphan Drug Designation for its OCU410ST (AAV5-*hRORA),* an adeno-associated virus serotype 5 capsid protein containing gene construct encoding human retinoic acid receptor-related orphan receptor alpha, for the "treatment of *ABCA4*-associated retinopathies" including Stargardt, Retinitis Pigmentosa 19 (RP19), and Cone-rod dystrophy 3 (CORD3) diseases.

"There are approximately 44,000 patients in the U.S. living with *ABCA4*-associated retinal diseases for whom no treatment options exist," said Dr. Shankar Musunuri, Chairman, CEO and Co-Founder of Ocugen. "This designation acknowledges the potential for OCU410ST to fulfill a significant unmet medical need and represents an important milestone in our effort to develop innovative treatments for inherited retinal diseases."

Orphan drug designation is granted by the FDA to certain products that show promise in the treatment, prevention, or diagnosis of rare and serious diseases affecting fewer than 200,000 people in the United States.

OCU410ST utilizes an AAV delivery platform for the retinal delivery of the *RORA* (RAR Related Orphan Receptor A) gene and represents Ocugen's modifier gene therapy approach, which is based on Nuclear Hormone Receptors (NHRs) that regulate diverse physiological functions such as photoreceptor development and maintenance, metabolism, phototransduction, inflammation and cell survival networks.

The *ABCA4* gene produces an ATP-binding cassette (ABC) superfamily transmembrane protein and expressed exclusively in retinal photoreceptors. It is involved in clearance of all-trans-retinal aldehyde, a byproduct of the retinoid cycle, from photoreceptor cells. Mutation in *ABCA4* gene results in Stargardt disease, a rare genetically inherited disease that directly affects the retina of the eye, often resulting in the slow progression of vision loss in children. In addition, different *ABCA4* alleles have been identified to cause other retinopathies such as CORD3 and RP19.

Ocugen plans to submit an IND in 2Q 2023 to initiate a Phase 1/2 clinical trial.

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 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
Tiffany.Hamilton@ocugen.com