



Ocugen Announces OCU400—Modifier Gene Therapy—Phase 1/2 Data Presentation at Retinal Cell and Gene Therapy Innovation Summit 2024

April 29, 2024

MALVERN, Pa., April 29, 2024 (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 Benjamin Bakall, MD, PhD, Director of Clinical Research at Associated Retina Consultants and Clinical Assistant Professor at the University of Arizona, College of Medicine—Phoenix will present data from the OCU400 Phase 1/2 clinical trial at the Retinal Cell and Gene Therapy Innovation Summit being held May 3, 2024 in Seattle, WA.

"I look forward to sharing more about my clinical experience with this novel modifier gene therapy with my peers and industry leadership during the summit," said Dr. Bakall. "There remains a significant unmet need for patients with retinitis pigmentosa (RP) and I believe that this approach can offer a new therapeutic option to address the disease itself."

The Retinal Cell and Gene Therapy Innovation Summit 2024 is jointly organized by the Foundation Fighting Blindness and the Oregon Health and Science University Casey Eye Institute. The Summit brings together representatives from the biotech and pharma industries, along with members of the medical and research communities, to discuss rapidly emerging ocular gene and cell therapies and strategize how to move the most advanced retinal disease therapies toward clinical utility.

Details on Dr. Bakall's presentation are as follows:

Presentation Title: "Nuclear Hormone Receptor-Based Gene Modifier Therapy: Safety and Efficacy from Phase 1/2 Clinical Trials for Retinitis Pigmentosa"

Date: Friday, May 3, 2024

Time: 10:50 – 11:05 a.m. (PT)

Location: Hyatt Regency Seattle

The OCU400 Phase 3 liMeliGhT clinical trial is currently underway and on track to meet the Company's 2026 BLA and MAA approval targets. Between the U.S. and EU, nearly 300,000 people are affected by RP.

About OCU400

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 drive 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 RP.

About RP

RP is a group of rare, genetic disorders that involve a breakdown and loss of cells in the retina, leading to vision loss and blindness. Currently, RP is associated with mutations in more than 100 genes. There are no approved treatment options that slow or stop the progression of multiple forms of RP. Proposed treatments for RP include gene replacement therapy, retinal implant devices, retinal transplantation, stem cells, vitamin therapy, and other pharmacological treatments. Current gene replacement therapies are promising but are limited to treating just a single mutation. In addition, while gene therapies may provide a new functional gene, they do not necessarily eliminate the underlying genetic defect, which may still cause stress and toxic effects leading to retina degeneration. Therefore, the development of gene-specific replacement therapy is highly challenging, especially when multiple and unknown genes are involved. Thus, novel therapeutic approaches targeting broader RP disease in a gene-agnostic manner offer greater hope for patients.

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 [X](#) 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, including, but not limited to, statements regarding qualitative assessments of available data, potential benefits, expectations for ongoing clinical trials, anticipated regulatory filings and anticipated development timelines, 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, including, but not limited to, the risks that preliminary, interim and top-line clinical trial results may not be indicative of, and may differ from, final clinical data; that unfavorable new clinical trial data may emerge in ongoing clinical trials or through further analyses of existing clinical trial data; that earlier

non-clinical and clinical data and testing of may not be predictive of the results or success of later clinical trials; and that that clinical trial data are subject to differing interpretations and assessments, including by regulatory authorities. 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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