Ocugen to Present on Modifier Gene Therapy Platform at Association for Research in Vision and Ophthalmology 2024 Annual Meeting

April 26, 2024

MALVERN, Pa., April 26, 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 the Company will present on its innovative modifier gene therapy platform, including OCU400 for the treatment of retinitis pigmentosa (Phase 3 LiMeliGhT clinical trial), OCU410 for the treatment of geographic atrophy (Phase 1/2 ArMaDa clinical trial), and OCU410ST for the treatment of Stargardt disease (Phase 1/2 GARDian clinical trial) at The Association for Research in Vision and Ophthalmology (ARVO) 2024 Annual Meeting in Seattle, WA from May 5-9, 2024.

“With three gene therapies to treat blindness diseases currently in the clinic, now is an exciting time for Ocugen and the patients who can potentially benefit from our first-in-class modifier gene therapy platform,” said Dr. Shankar Musunuri, Chairman, CEO and Co-founder of Ocugen. “We look forward to sharing more about the scientific foundation of our programs and providing clinical updates with industry leaders during ARVO.”

Ocugen’s presence at ARVO 2024 includes:

Paper Session (oral presentation)

Title: **OCU400 Nuclear Hormone Receptor-Based Gene Modifier Therapy: Safety and Efficacy from Phase 1/2 Clinical Trial for Retinitis Pigmentosa Associated with *NR2E3* and *RHO* Mutations**

Authors: Byron L. Lam, Arun K. Upadhyay, Shankar Musunuri, Murthy Chavali, Sahar Matloob, Nalin Mehta, David G. Birch, Paul Yang, Benjamin Bakall, Nieraj Jain, Jose S. Pulido, Borooah Shyamanga

Presenter: Byron Lam, MD, Professor of Ophthalmology, Dr. Mark J. Daily Endowed Chair, University of Miami
Presentation Number: 406
Location: Seattle Convention Center, Arch Building, Room 612
Date: Sunday, May 5, 2024
Time: 1:45-2 p.m. (PT)

Exhibitor Presentations

Title: **OCU400—A Gene Agnostic Modifier Gene Therapy for the Treatment of Retinitis Pigmentosa (Phase 1/2 Clinical Study Results and Phase 3 liMeliGhT Study Design)**
Presenter: Arun Upadhyay, PhD, Chief Scientific Officer, Head of Research & Development, Ocugen
Location: Show Floor, Educational Lounge, Booth #4921
Date: Monday, May 6, 2024
Time: 1 p.m. (PT)

Title: **OCU410 Gene Therapy—Multifactorial Therapeutic Intervention for Dry Age-Related Macular Degeneration**
Presenter: Huma Qamar, MD, MPH, Chief Medical Officer, Ocugen
Location: Show Floor, Educational Lounge, Booth #4921
Date: Tuesday, May 7, 2024
Time: 2 p.m. (PT)

Title: **Nuclear Hormone Receptor *RORA* as a Novel Modifier Approach for Treatment of Stargardt Disease**
Presenter: Murthy Chavali, PhD, Director, Clinical Development, Ocugen
Location: Show Floor, Educational Lounge, Booth #4921
Date: Wednesday, May 8, 2024
Time: 2 p.m. (PT)

Ocugen is committed to bringing game-changing therapies to treat inherited retinal diseases as well as blindness diseases affecting millions to market and working even harder to provide access to patients globally.

About AAV-*hNR2E3* (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 retinitis pigmentosa. Between the U.S. and EU, nearly 300,000 people are affected by retinitis pigmentosa. The OCU400 Phase 3 liMeliGhT clinical trial is currently underway and on track to meet the Company’s 2026 BLA and MAA filing targets.

About AAV-*hRORA* (OCU410/OCU410ST)

AAV-*hRORA* utilizes an AAV delivery platform for the retinal delivery of the *RORA* (ROR Related Orphan Receptor A) gene. The RORA protein plays an important role in lipid metabolism, reducing lipofuscin deposits and oxidative stress, and demonstrates an anti-inflammatory role as well as inhibiting the complement system in in-vitro and in-vivo (animal model) studies. These results demonstrate the ability to target multiple pathways linked with dry age-related macular degeneration and Stargardt pathophysiology. Ocugen is developing OCU410 as a one-time gene therapy for the treatment of GA (affecting one million people in the U.S.) and OCU410ST as a one-time gene therapy for the treatment of Stargardt disease (affecting 41,000 people in the U.S.).

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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