Ocugen Announces Compelling Preliminary Data for OCU410—a Single Dose Novel Modifier Gene Therapy to Treat Geographic Atrophy Secondary to Dry Age-Related Macular Degeneration

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MALVERN, Pa., Nov. 19, 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, biologics, and vaccines, today announced positive preliminary efficacy and safety data from the Phase 1 dose-escalation portion of the Phase 1/2 OCU410 ArMaDa clinical trial for geographic atrophy (GA), secondary to dry age-related macular degeneration (dAMD). Key findings include: no drug-related serious adverse events, reduced lesion growth, preservation of retinal tissue, and—most importantly—there was a positive effect on the functional visual measure of low luminance visual acuity (LLVA).

Currently, there are approximately three million people living with GA in the United States (U.S.) and Europe combined. Patients in the U.S. have only one option available, anti-complement therapy, which requires multiple injections and only addresses one aspect of the disease. There remains no treatment option for GA in Europe.

The OCU410 Phase 1 trial is evaluating nine patients in three dose cohorts (low, medium, and high). The following data was observed for the three patients in the low dose cohort at six months:

- Considerably slower lesion growth (21.4%) from baseline in treated vs. untreated fellow eyes that followed the natural history of the disease. This result is favorable when compared to published data on pegcetacoplan injected every month or every other month over six months.
- OCU410 treatment showed increasing preservation of retinal tissue around the GA lesions of treated eyes over six months, which also compared favorably to published data on pegcetacoplan given monthly and every other month.
- 100% of the OCU410 treated eyes showed stabilization of visual function demonstrating treatment benefit as measured by LLVA.

“Currently approved treatments for GA have not shown significant benefit in visual function. More importantly, we often do not realize the logistical challenge and emotional burden both patients and their caregivers must endure for every month or every other month visits,” said Syed M. Shah, MD, FACS, Director of Retina Service, Vice Chair for Research & Digital Health at Emplify Health – La Crosse, Wisconsin. “Based on the science and preliminary data, OCU410 has the potential to improve structural as well as functional outcomes. This ‘one-and-done’ treatment paradigm can be a gamechanger for how we treat patients with GA.”

“OCU410 addresses multiple aspects of the disease beyond the complement pathway,” said Dr. Huma Qamar, Chief Medical Officer at Ocugen. “The latest OCU410 data emphasizes the potential of novel modifier gene therapy as a one-time treatment for dAMD. We remain very encouraged by the latest safety and efficacy data and positive patient outcomes.”

Ocugen also announced promising data from the Phase 1/2 OCU410ST GARDian clinical trial for Stargardt disease and data on Leber congenital amaurosis (LCA) from the Phase 1/2 OCU400 clinical trial. All these findings, as well as commentary from study investigators and patient perspectives, were shared at the Company’s recent Clinical Showcase. The data affirms the potential for modifier gene therapy to address both rare inherited retinal diseases and blindness diseases affecting millions.

A full replay of the showcase is available on the Events section of the Ocugen website. For more information about Ocugen’s ongoing clinical trials, please contact clinical.request@ocugen.com

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 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, strategy, business plans and objectives for Ocugen’s clinical programs, plans and timelines for the preclinical and clinical development of Ocugen’s product candidates, including the therapeutic potential, clinical benefits and safety thereof, expectations regarding timing, success and data announcements of current ongoing preclinical and clinical trials, the ability to initiate new clinical programs; 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 annual and 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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