Data and Safety Monitoring Board Reviews Cohort 1 Safety Data and Approves Dosing Cohort 2 in the Clinical Trial of OCU200—a Novel Fusion Protein for Diabetic Macular Edema

March 18, 2025

- *OCU200 has a very favorable safety and tolerability profile*
- *No serious adverse events related to the study drug have been reported*
- *Dosing of the second cohort has been approved*

MALVERN, Pa., March 18, 2025 (GLOBE NEWSWIRE) -- Ocugen, Inc. (“Ocugen” or the “Company”) (NASDAQ: OCGN), a pioneering biotechnology leader in gene therapies for blindness diseases, today announced that the Data and Safety Monitoring Board (DSMB) for the OCU200 clinical trial recently convened and reviewed safety data following dosing of the first cohort in the dose-escalation portion of the Phase 1 study and approved continuation of dosing in the second cohort. OCU200 is a novel fusion protein consisting of two human proteins, tumstatin and transferrin, with the potential to treat diabetic macular edema (DME).

“OCU200 is given intravitreally,” said Peter Chang, MD, FACS, Co-President and Partner of the Massachusetts Eye Research and Surgery Institution (MERSI). “No serious adverse events related to OCU200 have been reported to date.”

The OCU200 Phase 1 clinical trial is a multicenter, open-label, dose-escalation study to assess drug safety via intravitreal injection in three cohorts: low dose (0.025 mg), medium dose (0.05 mg), and high dose (0.1 mg). All subjects will receive two doses six weeks apart, and patients will be followed for up to 6 months.

“It is encouraging that we have successfully completed dosing in the low dose cohort for OCU200, a novel biologic that has a very favorable safety and tolerability profile,” said Dr. Huma Qamar, Chief Medical Officer at Ocugen. “There remains a considerable unmet medical need for the 30% to 40% of DME patients who do not respond to current anti-VEGF therapies. OCU200 holds the promise of potentially benefiting all DME, diabetic retinopathy (DR), and wet age-related macular degeneration (wet AMD) patients.”

Approximately 12 million people in the United States and 130 million people worldwide are affected by DME, DR, or wet AMD. Patients affected by them share common symptoms, such as blurriness in vision and progressive vision loss, as the diseases progress. The formation of fragile and leaky new blood vessels leads to fluid accumulation in and around the retina, causing damage to vision.

OCU200 has the potential to change the treatment landscape for DME, DR, and wet AMD with its unique mechanism of action, binding the active component—tumstatin—to integrin receptors on active endothelial cells that play a crucial role in disease pathogenesis.

OCU200 brings an innovative biologic candidate to Ocugen’s ophthalmology portfolio targeting blindness diseases. The Company intends to complete the Phase 1 OCU200 clinical trial in the second half of 2025 and to provide preliminary safety and efficacy updates throughout the year.

About OCU200

OCU200 is a recombinant fusion protein that consists of two parts connected by a linker: tumstatin, the active component, acts as an anti-inflammatory, anti-VEGF agent by binding to integrin receptors; and transferrin, which targets the drug to the choroid and retina by binding transferrin receptors on endothelial cells. These features will potentially enable OCU200 to reduce the vascular permeability, inflammation, and neovascularization that drive the pathophysiology of DME, DR, and wet AMD at a significantly lower dose compared to currently approved therapies.

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 patients’ 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; the ability of OCU200 to perform in humans in a manner consistent with nonclinical or preclinical study 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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