Ocugen Announces European Medicines Agency Grants Orphan Medicinal Product Designation for Modifier Gene Therapy Candidate OCU410ST for Treatment of ABCA4-Associated Retinopathies including Stargardt Disease

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MALVERN, Pa., Nov. 20, 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 that the European Medicines Agency (EMA) has granted orphan medicinal product designation for OCU410ST for the treatment of *ABCA4*-associated retinopathies including Stargardt disease, retinitis pigmentosa 19 (RP19), and cone-rod dystrophy 3 (CORD3).

"We are deeply honored to receive orphan medicinal product designation from the EMA for OCU410ST. This recognition brings us one step closer to providing a much-needed option for Stargardt patients who currently have no therapies available," said Dr. Arun Upadhyay, Chief Scientific Officer and Head of R&D at Ocugen. "We are committed to advancing this treatment with urgency and dedication, with the hope of making a meaningful impact on the lives of those affected by this challenging disease."

The U.S. Food and Drug Administration (FDA) previously granted orphan drug designation to OCU410ST in April 2023. Stargardt disease affects approximately 100,000 people in the U.S. and Europe combined.

Orphan medicinal product designation in Europe offers certain benefits to drug developers while they develop drugs intended for safe and effective treatment, diagnosis, or prevention of rare diseases or conditions that impact fewer than 5 in 10,000 patients in the European Union. Benefits include protocol assistance, reduced regulatory fees, research grants, and 10 years of market exclusivity following regulatory approval.

Dosing in the first phase of the Phase 1/2 OCU410ST GARDian trial for Stargardt disease is complete and the Data and Safety Monitoring Board (DSMB) has recommended moving forward with Phase 2. To date, the safety and tolerability profile of OCU410ST appears to be very favorable.

Preliminary efficacy and safety data from the Phase 1 dose-escalation portion of the Phase 1/2 OCU410ST GARDian clinical trial was recently presented at Ocugen's Clinical Showcase. Data from evaluable subjects at six months demonstrated a remarkable 84% reduction in atrophic lesion growth in treated eyes versus untreated fellow eyes.

"We are encouraged by the preliminary efficacy data showing stabilization or improvement in visual function and retinal structure outcomes in OCU410ST treated eyes," said Dr. Huma Qamar, Chief Medical Officer at Ocugen. "These positive clinical and regulatory milestones continue to support the potential for OCU410ST to address inherited retinal diseases with a one-time therapy for life."

OCU410ST utilizes an AAV delivery platform for the retinal delivery of the *RORA* (RAR Related Orphan Receptor A) gene and further represents the impact of 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.

Ocugen intends to pursue an accelerated marketing authorization application (MAA) for OCU410ST.

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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