Ocugen Announces Phase 3 liMeliGhT Enrollment Completion for OCU400, a Novel Modifier Gene Therapy for Broad Retinitis Pigmentosa

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- *Enrollment for liMeliGhT, the first and largest gene therapy registrational trial for broad retinitis pigmentosa (RP) patients, was completed, reflecting strong interest from investigators and patients*
- *Topline Phase 3 data expected in 1Q 2027, advancing OCU400 towards potential approval in 2027 as a treatment option for early- to late-stage RP*
- *Positive long-term, 3-year Phase 1/2 durable, safety and tolerability data for OCU400 demonstrates sustained clinically meaningful, approximately 2-line LLVA gain, reinforcing durable gene-agnostic benefit*

MALVERN, Pa., March 02, 2026 (GLOBE NEWSWIRE) -- Ocugen, Inc. (Ocugen or the Company) (NASDAQ: OCGN), a pioneering biotechnology leader in gene therapies for blindness diseases, today announced that enrollment is now complete for the OCU400 Phase 3 liMeliGhT clinical trial for retinitis pigmentosa (RP). As a one-year clinical trial, topline data will be available in the first quarter of 2027. These data are anticipated to support the Biologics License Application (BLA) filing for OCU400 and potential approval in 2027. The European Medicines Agency (EMA) has also provided acceptability of the U.S.-based trial for submission of a Marketing Authorization Application (MAA).

“With enrollment complete for OCU400, we enter into a very significant time as a Company,” said Dr. Shankar Musunuri, Chairman, CEO, and Co-founder of Ocugen. “This milestone brings us even closer to potentially delivering our first novel modifier gene therapy candidate to market and providing a one-time treatment for life to hundreds of thousands of RP patients across the globe with unmet medical need.”

The liMeliGhT clinical trial enrolled 140 patients who were randomized 2:1 into the treatment group ($2.5\times^{10}$ vg per eye 250 µL) and untreated control group across mutations (*RHO* and gene-agnostic arms). The target population included patients with early- to late-stage disease among a broad RP population, including pediatrics (3+ years). The primary endpoint is 12-month change in visual function assessed by LDNA (luminance dependent navigation assessment) with improvement in Lux Level from baseline to 12 months. LDNA is a more sensitive and specific mobility test, proprietary to Ocugen.

“The Phase 3 liMeliGhT clinical trial includes representation of a wide range of gene mutations associated with early to advanced stages of RP and we believe the patient response will support the gene-agnostic mechanism of action of our novel modifier gene therapy platform,” said Dr. Huma Qamar, Chief Medical Officer of Ocugen. “I want to thank the investigators and clinical research teams for their tireless recruitment efforts and coordination among the trial sites to achieve the enrollment milestone. This dedication has the potential to shift the treatment paradigm for RP by targeting multiple genetic mutations with a single therapeutic approach.”

The OCU400 Phase 3 liMeliGhT clinical trial is the only broad RP gene-agnostic trial and the largest known Phase 3 orphan gene therapy trial.

“It is critical to work towards FDA-approved treatment options that address the significant gap that remains for the approximately 98% of people living with RP who are not candidates for the approved gene therapy for RP,” said Victor H. Gonzalez, Valley Retina Institute, McAllen, Texas, Faculty at University of Texas Rio Grande Valley and the Primary Investigator for the liMeliGhT clinical trial. “I am enthusiastic about the possibility of offering my patients with RP a safe, effective and durable treatment option that could potentially stabilize vision loss or improve vision.”

Positive long-term, 3-year Phase 1/2 data for OCU400 was recently assessed in evaluable subjects and builds on prior 2-year results showing consistent clinically meaningful, approximately 2-line LLVA gain across mutations. OCU400 maintained a favorable durability, safety and tolerability profile with no new treatment-related serious adverse events or adverse events of interest emerged. Additional data include:

- Visual function benefits were consistently observed over 3 years, with 88% (7/8) of evaluable treated subjects showing improvement or preservation versus untreated fellow eyes
- Approximately 2-line gain (N=8) observed across multiple mutation types in treated eyes compared to untreated eyes at 3 years

Enrollment completion in the OCU400 Phase 3 clinical trial combined with positive 3-year data from the Phase 1/2 study are important accomplishments in the Company’s plan to begin bringing this potential gene therapy to patients in 2027. Ocugen remains on track to file the rolling BLA in the third quarter of 2026.

About OCU400
OCU400 is the Company’s modifier gene therapy candidate based on a nuclear hormone receptor gene called *NR2E3*. This gene regulates diverse physiological functions within the retina, such as photoreceptor development and maintenance, metabolism, phototransduction, inflammation, and cell survival. Retinal cells in RP patients have a dysfunctional gene network, and OCU400 is designed to reset this network to reestablish a healthy cellular homeostasis—which has the potential to improve vision in patients with RP.

About RP
RP is a group of rare genetic disorders that cause a breakdown in the cells of the retina, leading to vision loss and blindness. 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 retinal degeneration. Therefore, the development of gene-specific replacement therapy will not address all forms of RP, especially when multiple and unknown genes are involved. Thus, novel therapeutic approaches targeting the broader RP disease in a gene-agnostic manner offer additional hope for patients.

About Ocugen, Inc.
Ocugen, Inc. is a biotechnology company focused on discovering, developing, and commercializing novel gene therapies to address major blindness diseases 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 address significant unmet medical need for large patient populations through our gene-agnostic approach. 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 OCU400 to perform in humans in a manner consistent with nonclinical, preclinical or previous clinical 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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