Ocugen, Inc. Announces Health Canada Approval to Initiate Phase 3 Clinical Trial for OCU400 – Modifier Gene Therapy for Broad Retinitis Pigmentosa Indication

August 26, 2024

MALVERN, Pa., Aug. 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 Health Canada provided a “No Objection Letter” to initiate the OCU400 Phase 3 liMeliGhT (pronounced “limelight”) clinical trial in Canada. OCU400 is a modifier gene therapy product candidate being developed for retinitis pigmentosa (RP).

“Expanding the clinical trial to Canada is significant as it will provide an opportunity to reach a broader patient population encompassing many gene mutations associated with RP,” said Dr. Shankar Musunuri, Chairman, CEO, and Co-Founder of Ocugen. “The Health Canada trial will run in parallel with the U.S. FDA trial, expediting the ability to potentially provide a gene-agnostic treatment option to approximately 110,000 patients in the United States (U.S.) and Canada.”

Currently there are approximately 10,000 patients in Canada with RP and 1.6 million patients globally. The Phase 3 study in Canada will enroll up to 50 subjects across a maximum of 5 sites for the liMeliGhT clinical trial.

Over 200 mutations in more than 100 genes have been linked to RP. The Phase 3 study, spanning one year, will enroll 150 participants divided into two study arms: 75 participants with *RHO* gene mutations and 75 participants who are gene agnostic. In each arm, participants will be randomized in a 2:1 ratio to receive either treatment (2.5×10^{10} vg/eye of OCU400) or remain in an untreated control group, respectively. The liMeliGhT study is recruiting patients aged eight and older, covering the full spectrum from early to late stages of RP progression.

An enhanced sensitive and specific measurement of functional vision test—Luminance Dependent Navigation Assessment (LDNA)—is the primary endpoint for the study. Specifically, the primary endpoint is a measurement of the change in functional vision from baseline to week 52 as measured by the ability of a study participant to navigate through a maze (the LDNA). Those who demonstrate an improved ability to navigate the maze in dimmer light (i.e., by ≥2 Lux levels) compared to baseline will be classified as "responders" to the therapy. The liMeliGhT study will focus on the proportion of responders in both the treated and untreated eyes.

“Establishing clinical sites in Canada may expedite recruitment and open doors for broader commercialization with the U.S. and Europe,” said Dr. Huma Qamar, Chief Medical Officer at Ocugen. “With only one currently approved treatment targeting a single mutation associated with RP, there remains a significant unmet medical need, and patients worldwide are eager for new therapeutic options. It is highly rewarding to extend our efforts into a new region and offer hope to Canadian patients with RP.”

Ocugen previously announced that OCU400 has received orphan drug and RMAT designations from the FDA. OCU400 remains on track for the 2026 BLA and MAA approval targets.

About OCU400

OCU400 is the Company’s modifier gene therapy product 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 resets 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 greater hope for patients.

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.

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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