



Ocugen Gains FDA Alignment on Key Aspects of OCU400—Modifier Gene Therapy—Pivotal Phase 3 Study Design

December 21, 2023

MALVERN, Pa., Dec. 21, 2023 (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 the Company received alignment from FDA on key aspects of the Phase 3 clinical trial design to assess the safety and efficacy of OCU400 in patients with *RHO* and other gene mutations associated with Retinitis Pigmentosa (RP).

"This news brings us even closer to fulfilling our mission to bring our first-in-class, gene-agnostic therapies to market and provide access to patients globally," said Dr. Shankar Musunuri, Chairman, Chief Executive Officer, and Co-Founder of Ocugen. "We look forward to beginning the Phase 3 clinical trial, which we plan to initiate in early 2024."

During a multidisciplinary meeting with FDA, based on preliminary results from an ongoing Phase 1/2 study, Ocugen received alignment on key aspects of the Phase 3 study design—including the study endpoint, patient enrollment strategy, and study duration of one year. The Phase 3 clinical trial will enroll a broader group of RP patients, including patients with the most common *RHO* gene mutation, based on OCU400's potentially gene-agnostic mechanism of action.

With orphan drug and RMAT designations in place for OCU400, FDA's alignment on key aspects of the Phase 3 study design positions Ocugen to confidently move forward in pursuing product development and licensure for OCU400.

Currently there are approximately 110,000 patients in the United States with RP and 1.6 million patients globally. Of these patients, more than 10% have the *RHO* genetic mutation. Advancing OCU400 to Phase 3 clinical development will be an important step toward addressing unmet needs in the RP patient community.

About Ocugen, Inc.

Ocugen, Inc. is a biotechnology company focused on discovering, developing, and commercializing novel gene and cell therapies 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, statements regarding qualitative assessments of available data, potential therapeutic and clinical benefits of our product candidates, expectations for clinical trial timing and results, anticipated timing of clinical trial updates and expectations for timing and outcome of regulatory interactions, 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; that that clinical trial data are subject to differing interpretations and assessments, including by regulatory authorities; that receipt of orphan drug and RMAT designations may not lead to faster development or regulatory review; and that regulatory authorities may disagree with additional aspects of our clinical trial designs or may not approve our future IND applications on the anticipated timeline or at all. 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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