Ocugen to Discontinue Phase 3 oGVHD Trial

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Breakthrough Modifier Gene Therapy platform continues to advance towards clinic

MALVERN, Pa., June 01, 2020 (GLOBE NEWSWIRE) -- Ocugen, Inc. (NASDAQ: OCGN), a clinical-stage company focused on discovering, developing and commercializing transformative therapies to treat the whole eye, today announced the decision to discontinue the Phase 3 trial of OCU300 for ocular Graft vs. Host Disease (oGVHD). The decision to stop the trial is based on results of a pre-planned interim sample size analysis conducted by an independent Data Monitoring Committee, which indicated the trial was unlikely to meet its co-primary endpoints upon completion. The study was not stopped based on safety concerns. We will analyze the full data when it is available.

"This is disappointing news, especially for those who suffer from oGVHD. Our hope was to provide the first treatment for this complex, orphan disease. We are grateful to the patients, their families and the investigators who participated in our clinical trial," said Shankar Musunuri, Chairman, CEO and Co-Founder of Ocugen. "We are committed to advancing our transformative therapies and will shift our resources to focus on our breakthrough modifier gene therapy platform and novel biologic programs for patients suffering from blindness diseases. We remain on track to enter the clinic by next year with our first gene therapy product candidate, OCU400, which targets inherited retinal diseases. Our additional pipeline products, OCU410 and OCU200, which are focused on major retinal diseases, are targeted to enter the clinic by 2022. As a result of discontinuing this study and proceeds from our ATM program we believe that we have sufficient cash to fund our current operations into the fourth quarter of 2020.

About OCU400

Ocugen is developing a modifier gene therapy platform to generate therapies designed to fulfill unmet medical needs in the area of retinal diseases, including inherited retinal diseases ("IRDs"). This modifier gene therapy platform is based on nuclear hormone receptors ("NHRs"), which have the potential to restore homeostasis, the basic biological processes in the retina and potentially rescue photoreceptors from degeneration. This technology was licensed from the Harvard Medical School. Unlike single-gene replacement therapies, which only target one gene at a time, we believe OCU400, through its use of NHRs, represents a novel approach in that it may address multiple retinal diseases with one product. Consisting of a functional copy of the nuclear hormone receptor gene *NR2E3*, OCU400 is delivered to target cells in the retina using an adeno-associated viral (AAV) vector. OCU400 has received two Orphan Drug Designations from the Food and Drug Administration, one for the treatment of *NR2E3* mutation-associated retinal diseases and the other for the treatment of *CEP290* mutation-associated retinal diseases. OCU400 has the potential to eliminate the need for developing more than 150 individual products and provide one treatment option for all Retinitis Pigmentosa (RP) patients.

About Ocugen, Inc.

Ocugen, Inc. is a clinical-stage biopharmaceutical company focused on discovering, developing and commercializing transformative therapies to treat the whole eye. Our breakthrough modifier gene therapy platform has the potential to treat multiple retinal diseases with one drug – "one to many" and our novel biologic product candidate aims to offer better therapy to patients with underserved diseases such as wet age-related macular degeneration, diabetic macular edema and diabetic retinopathy. For more information, please visit www.ocugen.com.

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, 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. These and other risks and uncertainties are more fully described in our periodic filings with the Securities and Exchange Commission (the "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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