Ocugen Receives Orphan Designation for OCU100 to Treat Retinitis Pigmentosa

June 5, 2014

Licensed Two Biological Drug Candidates from University of Colorado

AURORA, Colo.--(BUSINESS WIRE)--Ocugen, Inc. and the University of Colorado today announced exclusive license agreements that allow for Ocugen to continue developing two drug candidates for the treatment for ophthalmology indications, and that one of the assets, OCU100, recombinant lens epithelium derived growth factor 1-326 (LEDGF1-326), received orphan-drug status from the U.S. Food and Drug Administration for treatment of retinitis pigmentosa (RP), a rare eye disease.

“Orphan drug designation from the FDA’s Office of Orphan Products Development is a significant milestone that will allow Ocugen to accelerate the clinical development of OCU100, which has the potential to be the first approved therapeutic for retinitis pigmentosa,” said Shankar Musunuri, PhD, MBA, founder and chairman of the Ocugen Board of Directors.

Ocugen scientific founder and board member Uday Kompella, PhD, a professor of Pharmaceutical Sciences, Ophthalmology and Bioengineering at CU’s Anschutz Medical Campus, is the inventor of OCU100. Ocugen licensed all assets related to LEDGF, including LEDGF1-326 and OCU200, an anti-angiogenic tumstatin fusion protein, to be developed for treatment of wet age-related macular degeneration (AMD) from the University of Colorado in March.

Retinitis pigmentosa is a rare eye disease caused by inherited gene mutations that lead to retinal degeneration affecting approximately 100,000 people in the United States, according to the Foundation Fighting Blindness. People with RP experience a gradual decline in their vision because photoreceptor cells in the retina die. It is a progressive disorder, and most people with RP are legally blind by age 40. There is no FDA approved therapeutic for RP.

“OCU100 has shown the potential as a promising therapeutic agent for treating retinitis pigmentosa by reducing protein aggregation and associated cellular stresses, which are known to contribute to this condition,” said Dr. Kompella. “With impressive preclinical data, we look forward to progressing with a phase 1 study for safety and tolerability in patients sometime in 2015.”

Dr. Kompella said a variety of mutations, including P23H mutation in rhodopsin, a critical protein in the retina that is responsible for vision, have been linked to the development of RP. P23H rhodopsin is known to form large clusters or aggregates within retinal cells, leading to cellular stress and ultimately cell death. OCU100 with its ability to rescue retinal cells from protein aggregation and associated stresses, has the potential to revolutionize the treatment of RP.

About Orphan Drug Designation

FDA Office of Orphan Products Development (OOPD) grants orphan designation for novel drugs or biologics that treat a rare disease or condition affecting fewer than 200,000 patients in the U.S. Orphan designation qualifies the sponsor of the drug for various development incentives of the Orphan Drug Act (ODA) including seven-year period of U.S. marketing exclusivity, tax credits for qualified clinical testing, waiver of prescription drug user fee for marketing application, and ability to apply for grants. The OOPD also works on rare disease issues with the medical and research communities, professional organizations, academia, governmental agencies, industry, and rare disease patient groups.

About Ocugen, Inc.

Ocugen is advancing novel biologicals discovered based on endogenous proteins with well understood biology at the molecular, cellular, and whole animal level to treat eye diseases. The therapeutic proteins in the pipeline are derived from cell survival factors such as lens epithelium derived growth factor (LEDGF) and anti-angiogenic proteins such as tumstatin. The intellectual property covers a variety of related protein constructs including fusion proteins with superior activity.

About the University of Colorado

The University of Colorado is a premier public research university with four campuses: the University of Colorado Boulder, the University of Colorado Colorado Springs, the University of Colorado Denver and the University of Colorado Anschutz Medical Campus. Some 57,591 students are pursuing academic degrees at CU. Academic prestige is marked by the university’s five Nobel laureates, eight MacArthur “genius” Fellows, 18 alumni astronauts and 19 Rhodes Scholars. For more information about the entire CU system, and to access campus resources, go to www.cu.edu.

Contacts

Media Contact:
David Connolly, 617-374-8800 ext 108
LaVoieHealthScience
dconnolly@lavoiehealthscience.com
or
University of Colorado Contact:
Lindsay Lennox, 303-735-5518
University of Colorado Technology Transfer Office
lindsay.lennox@cu.edu