BIOWORLD™ TODAY
THE DAILY BIOPHARMACEUTICAL NEWS SOURCE
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JUNE 6, 2014
VOLUME 25, NO. 109
SPECIAL REPRINT

NewCo News: Start-up Ocugen eyes new opthalmology opportunities

By Michael Fitzhugh, Staff Writer

Entrepreneur Shankar Musunuri's newest venture, Ocugen Inc., is jumping into the race to develop a treatment for the rare disease retinitis pigmentosa (RP) and another therapy for wet age-related macular degeneration (AMD), with two preclinical biologics exclusively licensed from the University of Colorado.

Musunuri, co-founder and former CEO of Nuron Biotech Inc., is now president and CEO of Iogenetics Inc. He co-founded Aurora, Colo.-based Ocugen late last year. As chairman of the fledgling company, he's putting development of OCU100, the company's lead candidate, into motion and recruiting an executive team to lead the work. Meanwhile, with FDA orphan status for the drug already in hand, Musunuri told *BioWorld Today* that OCU100 could enter phase I testing for RP in late 2015, assuming the company can secure financing.

If successful, OCU100 could provide the first approved drug for some of the 100,000 people in the U.S. with the rare inherited eye disease. People with RP experience a gradual decline in their vision as the photoreceptor cells in the retina die.

Most people with the condition are legally blind by age 40. While medical need in the area is high, so is development interest, with 33 active phase I, II and III trials, according to the Cortellis Clinical Trials Intelligence database.

Ocugen's other co-founder and board member, Uday Kompella, invented both drugs. A former classmate of Musunuri's, the CU professor took the academic path, focusing on drug delivery and opthalmology, while Musunuri honed his business chops at Pfizer Inc. and Wyeth.

The two kept in touch and, when Kompella reached out to share his inventions with Musunuri, they decided to found Ocugen to develop the drugs.

After what Musunuri said was an easy and efficient negotiation with CU, in March Ocugen finished licensing assets for the recombinant lens epithelium-derived growth factor 1-326, now called OCU100, and an anti-angiogenic tumstatin fusion protein, now OCU200.

A variety of mutations, including P23H mutation in rhodopsin, an important protein in the retina, have been linked to the development of RP, Kompella said. "P23H rhodopsin is known to form large clusters or aggregates within retinal cells, leading to cellular stress and ultimately cell death," he said.

OCU100 can "rescue" retinal cells from protein aggregation and the stresses that aggregation poses.

Though Musunuri declined to say how much Ocugen would need to advance the drug, Sucampo Pharmaceuticals Inc. last year secured up to $22 million, which it said would cover the majority of development costs for its unrelated RP drug. That phase III program, testing the already-approved eye drug unoprostone Rescula (isopropyl) for the new indication, is expected to yield top-line results in early 2015.

Ocugen's second candidate, OCU200, has shown early promise in treating wet AMD, a disease that leads to blindness for the majority of the nearly 600,000 cases diagnosed globally each year.

Musunuri provided some of the seed funding for Ocugen himself, he said.

As he begins the journey to finance the company further, he'll likely be helped by growing investor interest in advanced opthalmology therapies. Optogenetics is taking off as researchers and investors take a closer look at techniques to deliver genes or chemicals to activate neurons in response to light.

Genable Technologies Ltd., for instance, recently tapped Philadelphia-based Spark Therapeutics LLC to provide manufacturing services and clinical development assistance on its preclinical lead program, GT038 for rhodopsin-linked autosomal dominant RP. (See *BioWorld Today*, Sept. 30, 2013, and March 26, 2014.) //