Ocugen
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Meeting patient needs through courageous innovation

Pennsylvania-based Ocugen is a fully integrated, patient-centric biotech company focused on the development of vaccines in support of public health, and gene and cell therapies targeting unmet medical needs, all based on its three scientific platforms.

Founded in 2013 by CEO and chair Shankar Musunuri and the University of Colorado's Uday Kompella, Ocugen is dedicated to developing first-in-class gene therapies for eye diseases that lead to blindness, and regenerative cell therapies for serious conditions such as articular cartilage lesions, both areas that lack effective treatment options. The company has three platforms: modifier gene therapy; regenerative cell therapy; and an inhaled mucosal vaccine platform. However, for Ocugen, it's about more than commercializing technologies in major markets.

"We know that gene and cell therapies are costly. Patients in lower income countries should not be denied access to treatments. We will do everything to get market access for people across the globe," said Musunuri.

Keeping a focus on eye disease

"Vision is critical to experiencing life fully, and developing products that have the potential to retain or restore sight is very powerful," said Arun Upadhyay, CSO and Head of Research, Development and Medical.

Retinitis pigmentosa (RP) affects around 1.5 million people worldwide and is the leading cause of visual disability in people aged over 60. Leber congenital amaurosis (LCA) is one of the most common causes of blindness in children, with about 180,000 cases worldwide. There are more than 125 mutated genes associated with these rare genetic diseases, with no available cure.

Unlike single gene approaches, Ocugen's gene therapies for eye diseases target master genes rather than individual genetic mutations (Fig. 1).

"Our approach aims to carry out a molecular reset of the health and survival gene networks in the eye, which are perturbed and imbalanced in disease state, to restore retinal cell homeostasis and create a healthy environment for the retinal cells' survival and function," said Upadhyay. "By targeting master genes rather than the diverse specific gene mutations, we are creating a mutation-agnostic treatment that could have a broader application, with a single, potentially curative injection."

OCU400, for RP and LCA, is Ocugen's lead gene therapy. Currently in phase 1/2 trials, preliminary data showed a positive trend in visual function based on a multi-luminance mobility test (MLMT) and visual acuity. A phase 3 trial for adult and pediatric RP and LCA patients is planned for late 2023 or early 2024.

Age-related macular degeneration (AMD) affects more than 266 million people worldwide, and around 90% of these have the dry form of the disease. AMD is a multifactorial disorder with a genetic component.

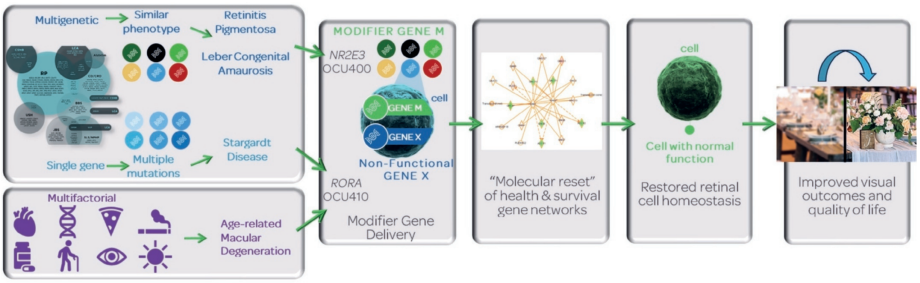

Fig. 1 | Mechanism of action of modifier gene therapy approach in inherited retinal disorder (retinitis pigmentosa, Leber congenital amaurosis and Stargardt) and multifactorial complex disease (dry AMD).

Current treatments include small molecules or biologics that need to be administered lifelong, making compliance a challenge; gene therapy has potential to be a once-for-all treatment. OCU410 targets multiple pathways disrupted in the pathogenesis of dry AMD, such as the complement system, inflammatory cascades, lipid metabolism, and oxidative stress. The current standard-of-care only targets complement, requires multiple injections per year, and has reported side effects.

"Once lesions are present in dry AMD, there are few clinical options," said Upadhyay. "Like our other gene therapies, our gene therapy for dry AMD is a gene modifier approach, so it has the potential to tackle a number of the pathways that cause the disease, and consequently fosters homeostasis—a balanced stable physiological state."

The United States Food and Drug Administration (FDA) has cleared investigational new drug (IND) applications for OCU410 for dry AMD and OCU410ST for Stargardt disease, an orphan indication, and expects to begin a clinical trial by the end of 2023.

Targeting regenerative medicine

Knee cartilage damage and degeneration can be managed through pain relief, or treated by surgical repair, autologous cartilage transplantation or joint replacement. The currently marketed product uses autologous cells loaded on collagen membrane.

"The current approach uses a two-dimensional scaffold. Our approach creates three-dimensional tissue, growing the patient's knee cartilage cells in bioreactors under conditions that mimic the pressure variations and oxygen level experienced in the joint. This allows the cells to mature and secrete extracellular matrix components crucial for cartilage function and be implanted as soon as six to eight weeks, and integrate more quickly after transplant," said Musunuri.

"Our goal is to launch our gene therapy OCU400 in 2026, with at least two gene therapies and one cell therapy launched by 2028," said Musunuri. "We will continue to work with the FDA, EMA [European Medicines Agency] and other agencies and are hoping for parallel approvals."

Ocugen is also developing an inhalation vaccine platform for COVID-19 and flu based on a novel ChAd platform. The vaccines are designed to provide durable immune response for up to one year, reducing transmission and protecting against new variants.

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Global access through partnerships

Ocugen is seeking corporate partners with a shared vision and the right infrastructure to support global launches. "For our ophthalmologic gene therapies, we are looking for partners who have strong networks with relevant health care professionals, medical centres and payers, and commercial experience in the field. This may mean we need a number of regional partners. We are proud of our first-in-class platform technologies, and our success will be defined by the number of patients that are treated," concluded Musunuri.

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