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Case Western Reserve University and Akouos sign exclusive licensing agreement to advance development of gene therapy technology as a potential treatment for a type of Usher syndrome

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[Case Western Reserve University \(https://case.edu/\)](https://case.edu/) and Boston-based [Akouos \(https://www.akouos.com/\)](https://www.akouos.com/) have entered into an exclusive licensing agreement to develop a patented gene therapy with the potential to treat hearing loss associated with a type of Usher syndrome, the most common deaf-blind disorder.

Usher syndrome is characterized by partial or total hearing loss and vision loss that worsens over time, according to the National Institutes of Health (NIH). The hearing loss is classified as “sensorineural,” or caused by abnormalities of the inner ear. The vision loss is due to the degeneration of light-sensitive tissue (retina) at the back of the eye. Usher syndrome is subdivided into various subtypes and each subtype is associated with a single gene defect.

According to NIH, Usher syndrome affects about four to 17 per 100,000 people nationally and accounts for about half of all hereditary deaf-blindness cases. The condition accounts for 3% to 6% percent of children who are deaf, and another 3% to 6% of children who are hard-of-hearing.

The technology, jointly owned by Case Western Reserve and University Hospitals (UH), is based on research from the lab of [Kumar Alagramam \(https://casemed.case.edu/Otolaryngology/faculty/alagramam.php\)](https://casemed.case.edu/Otolaryngology/faculty/alagramam.php), the Anthony J. Maniglia Chair for Research and Education, professor of otolaryngology, genetics and genomic sciences, and neurosciences at the Case Western Reserve School of Medicine and director of research at the [Ear, Nose & Throat Institute \(https://www.uhhospitals.org/services/Ear-Nose-and-Throat-Services\)](https://www.uhhospitals.org/services/Ear-Nose-and-Throat-Services) at UH.

“The technology allowed us to develop an even more precise animal model of hearing loss associated with Usher syndrome type 3A (USH3A) than previously reported, and it revealed the potential for the technology to deliver preservation of hearing and quality of life for children and adults diagnosed with the genetic disorder,” Alagramam said. “Further, lessons learned from this technology could be applied to gene therapy as a potential treatment for other forms of inherited hearing loss.”

The gene therapy may stop the progression of hearing loss and prevent deafness in people with USH3A, a form of hereditary hearing loss linked to defects in the sensory “hair” cells in the inner ear.

Akouos, a precision genetic medicine company founded in 2016 to develop gene therapies that restore and preserve hearing, licensed the rights to commercialize the technology in the United States, following additional development efforts and pending demonstration of safety and efficacy in future clinical trials.

“At Akouos, we value working in close collaboration with pioneering investigators and institutions to advance precision medicines for individuals and families in search of hearing loss treatment,” said Manny Simons, founder, president and CEO of Akouos. “With no FDA-approved medicines available for the millions of individuals worldwide with genetically-driven hearing loss or deaf-blind disorders, it is imperative that companies, academic research institutions, and advocacy organizations work together and accelerate toward creative solutions. Our collaboration with Dr. Alagramam and Case Western Reserve aims to advance the field of precision genetic medicines for deaf-blind disorders, such as Usher syndrome, and deepen our understanding of the genetics driving this and other rare hearing disorders.”

“Partnering with Akouos provides an excellent opportunity for us to translate this to the first potential therapy to prevent hearing loss caused by Usher syndrome,” said Stephanie Weidenbecher, senior licensing manager in Case Western Reserve’s Technology Transfer Office.

“We appreciate the opportunity to partner with CWRU and Akouos to commercialize this technology that will bring hope to patients impacted by hearing loss due to USH3A,” said Neil Wyant, managing director of UH Ventures, the innovation and commercialization arm of UH.

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