AKOUOS DISCLOSES LEAD PROGRAM, AK-OTOF, A POTENTIAL GENE THERAPY FOR SENSORINEURAL HEARING LOSS

September 12, 2019

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Preclinical Data to be Presented this Weekend at the American Neurotology Society Annual Meeting

Boston, Mass. – September 12, 2019 – Akouos, a precision genetic medicine company developing gene therapies to restore and preserve hearing, today disclosed its lead program, AK-OTOF, a gene therapy focused on restoring hearing in individuals with sensorineural hearing loss due to mutations in the otoferlin (OTOF) gene.

Normal otoferlin function enables the sensory cells of the ear (hair cells) to release neurotransmitter in response to stimulation by sound to activate auditory neurons. Without functional otoferlin protein, auditory signals received by the ear cannot be transmitted to the brain. AK-OTOF uses an adeno-associated viral (AAV) vector to deliver a healthy copy of the OTOF gene to cochlear hair cells, with the goal of restoring long-term physiologic hearing following a single administration to the inner ear. AK-OTOF is intended to treat individuals with sensorineural hearing loss due to mutations in the OTOF gene, who typically have severe hearing loss in both ears from birth, by promoting the expression of normal, functional otoferlin protein in affected cells of the cochlea. Mutations in the OTOF gene are reported to be a major cause of genetic hearing loss, affecting an estimated 200,000 individuals worldwide.

“The Akouos team has made great progress building the leading precision genetic medicine platform focused on inner ear disorders, and we are proud to announce our first gene therapy candidate from that platform, AK-OTOF,” said Manny Simons, Ph.D., founder, president and CEO of Akouos. “Together with leading scientists and clinicians around the world, we are working with urgency to advance AK-OTOF for individuals with sensorineural hearing loss due to mutations in the otoferlin gene. We have already begun interacting with the U.S. Food and Drug Administration about our IND-enabling studies to support first-in-human clinical trials, and we will provide an update on commencement of our first clinical trial as soon as possible.”

Akouos co-founder and chief medical officer, Michael McKenna, M.D., will present preclinical data on AK-OTOF, as well as other Akouos updates, during the Frank M. Rizer Memorial Lecture at the 54th Annual American Neurotology Society Fall Meeting. The presentation, “Emerging Role for Gene Therapy in the Treatment of Sensorineural Hearing Loss,” will take place on Saturday, September 14, 2019 at 2:25 p.m. CT in New Orleans.

About Sensorineural Hearing Loss
Sensorineural hearing loss results from dysfunction or damage to sensory cells and/or nerve fibers of the inner ear. Sensorineural hearing loss is found in most cases of newborn deafness and affects nearly a quarter of all adults over the age of 65, making it the most common form of hearing loss and one of the most common of all sensory disorders. Akouos is initially focused on monogenic forms of sensorineural hearing loss, in which mutations in individual genes lead to profound deafness.

About Akouos
Akouos is a precision genetic medicine company dedicated to developing gene therapies that restore and preserve hearing. Leveraging its adeno-associated viral (AAV) vector-based gene therapy platform, Akouos is focused on developing precision therapies for forms of sensorineural hearing loss. Headquartered in Boston, the Company was founded in 2016 by world leaders in the fields of neurotology, genetics, inner ear drug delivery, and AAV gene therapy. Akouos has strategic partnerships with Massachusetts Eye and Ear Infirmary and Lonza, Inc. For more information, please visit www.akouos.com.

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