Akouos Receives Orphan Drug and Rare Pediatric Disease Designations for AK-OTOF for the Treatment of Otoferlin Gene-Mediated Hearing Loss

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BOSTON, April 13, 2021 (GLOBE NEWSWIRE) -- Akouos, Inc. (NASDAQ: AKUS), a precision genetic medicine company dedicated to developing potential gene therapies for individuals living with disabling hearing loss worldwide, today announced that the U.S. Food and Drug Administration (FDA) has granted both Orphan Drug Designation (ODD) and Rare Pediatric Disease Designation (RPDD) for AK-OTOF, a gene therapy intended for the treatment of otoferlin gene-mediated hearing loss. Akouos anticipates that it will submit an investigational new drug application (IND) for AK-OTOF in the first half of 2022.

Otoferlin gene (*OTOF*)-mediated hearing loss is a form of sensorineural hearing loss caused by mutations in the *OTOF* gene. The *OTOF* gene encodes otoferlin, a protein that enables the inner hair cells of the cochlea to release neurotransmitter vesicles in response to stimulation by sound to activate auditory neurons. Most individuals with *OTOF*-mediated hearing loss have Severe-to-Profound sensorineural hearing loss from birth and approximately 20,000 individuals are affected in the United States and Europe. AK-OTOF is designed to treat the underlying cause of *OTOF*-mediated hearing loss through delivery of a transgene using a dual vector technology that results in expression of normal, functional otoferlin protein in the affected cells, namely inner hair cells, in the cochlea.

“There are currently no pharmacologic treatment options for individuals with *OTOF*-mediated hearing loss, or for any other form of sensorineural hearing loss. The nonclinical data reported to date for AK-OTOF demonstrate durable recovery of auditory function and support future clinical development.” said Jen Wellman, chief operating officer of Akouos. “We believe these are the first Orphan Drug and Rare Pediatric Disease designations granted by FDA for a genetic form of hearing loss, and represent an important milestone for the field of inner ear genetic medicines. The receipt of both designations could help us accelerate development of AK-OTOF, a therapy that we believe has the potential to restore physiologic hearing and provide long-lasting benefits to these individuals and their families.”

The Office of Orphan Products Development of FDA grants ODD to drugs and biologics intended for the treatment, diagnosis, or prevention of rare diseases, or conditions affecting fewer than 200,000 people in the United States. The designation affords Akouos the potential for certain benefits, including up to seven years of post-approval market exclusivity, assistance in the drug development process, tax credits for clinical development, and exemptions from certain FDA fees.

Rare pediatric disease designation is granted by FDA to encourage development of treatments for serious or life-threatening rare diseases in which the disease manifestations primarily affect individuals aged from birth to 18 years. Under the Priority Review Voucher program, and subject to FDA approval of AK-OTOF for the treatment of *OTOF*-mediated hearing loss, Akouos may be eligible to receive one priority review voucher, which could then be redeemed to receive priority review for a subsequent marketing application for a different product or sold or transferred to another sponsor.

About Akouos

Akouos is a precision genetic medicine company dedicated to developing gene therapies with the potential to restore, improve, and preserve high-acuity physiologic hearing for individuals living with disabling hearing loss worldwide. Leveraging its precision genetic medicine platform that incorporates a proprietary adeno-associated viral (AAV) vector library and a novel delivery approach, Akouos is focused on developing precision therapies for forms of sensorineural hearing loss. Headquartered in Boston, Akouos was founded in 2016 by leaders in the fields of neurotology, genetics, inner ear drug delivery, and AAV gene therapy.

Forward-Looking Statements

Statements in this press release about future expectations, plans and prospects, as well as any other statements regarding matters that are not historical facts, may constitute “forward-looking statements” within the meaning of The Private Securities Litigation Reform Act of 1995. These statements include, but are not limited to, statements relating to the timing of our IND submission for AK-OTOF and the potential receipt of a priority review voucher and other benefits from the ODD and RPDD. The words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “will,” “would,” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: our limited operating history; uncertainties inherent in the development of product candidates, including the initiation and completion of nonclinical studies and clinical trials; whether results from nonclinical studies will be predictive of results or success of clinical trials; the timing of and our ability to submit applications for, and obtain and maintain regulatory approvals for, our product candidates; our expectations regarding our regulatory strategy; our ability to realize the expected benefits of ODD and RPDD; the potential for changes in regulatory requirements; our ability to fund our operating expenses and capital expenditure requirements with our cash, cash equivalents, and marketable securities; the potential advantages of our product candidates; the rate and degree of market acceptance and clinical utility of our product candidates; our estimates regarding the potential addressable patient population for our product candidates; our commercialization, marketing, and manufacturing capabilities and strategy; our ability to obtain and maintain intellectual property protection for our product candidates; our ability to identify additional products, product candidates, or technologies with significant commercial potential that are consistent with our commercial objectives; the impact of government laws and regulations; risks related to competitive programs; the potential that our internal manufacturing capabilities and/or external manufacturing supply may experience delays; the impact of the COVID-19 pandemic on our business, results of operations, and financial condition; our ability to maintain and establish collaborations or obtain additional funding; and other factors discussed in the “Risk Factors” section included in the Company’s Annual Report on Form 10-K for the fiscal year ended December 31, 2020 filed with the Securities and Exchange Commission, and in other filings that the Company makes with the Securities and Exchange Commission in the future. Any forward-looking statements contained in this press release speak only as of the date hereof, and the Company expressly disclaims any obligation to update any forward-looking statement, whether as a result of new information, future events or otherwise.

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