Akouos Announces European Commission Designation of AK-OTOF for the Treatment of Otoferlin Gene-Mediated Hearing Loss as an Orphan Drug

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BOSTON, Aug. 11, 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 European Medicines Agency (EMA) Committee for Orphan Medicinal Products (COMP) issued a positive opinion on the company's application for orphan drug designation for AK-OTOF, a gene therapy intended for the treatment of otoferlin gene-mediated hearing loss. The positive opinion was subsequently adopted by the European Commission. AK-OTOF was previously granted Orphan Drug Designation (ODD) and Rare Pediatric Disease Designation (RPDD) by the U.S. Food and Drug Administration for this same indication.

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.

"Severe to Profound sensorineural hearing loss from birth caused by mutations in the *OTOF* gene is a high unmet need, especially given that there are currently no approved pharmacologic treatment options available," said Katie Wachtel, M.S. and vice president of regulatory affairs of Akouos. "The orphan drug designation granted by the European Commission is an important step towards advancing the global development of AK-OTOF, a gene therapy with potential to restore physiologic hearing and provide long-lasting benefit to individuals with *OTOF*-mediated hearing loss. Along with the ODD and RPDD designations previously granted by FDA, the orphan drug designation for AK-OTOF in the European Union could accelerate our development of AK-OTOF and our progress towards our mission of healthy hearing available to all."

Orphan drug designation in the European Union is granted by the European Commission based on an opinion issued by the EMA COMP. An orphan drug designation provides a number of benefits, including fee reductions, regulatory assistance, and the possibility to apply for a centralized European Union marketing authorization. Marketing authorization for an orphan drug can lead to a ten-year period of market exclusivity.

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.

Cautionary Note Regarding 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 initiation, plans, and timing of our future clinical trials and our research and development programs, and the potential receipt of exclusivity and other benefits from Orphan Drug Designation in the European Union and Orphan Drug Designation and Rare Pediatric Disease Designation in the United States. 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 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 and any changes in such 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" included in the Company's Quarterly Report on Form 10-Q for the three months ended March 31, 2021 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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