



**Akouos to Build Premier Gene Therapy Company Focused on Hearing and Balance Disorders with Backing from Leading Life Science Investors and Strategic Licenses from Massachusetts Eye and Ear and Lonza**

- Akouos raises \$7.5 million in equity financing from 5AM Ventures, New Enterprise Associates (NEA), and Partners Innovation Fund.
- Massachusetts Eye and Ear and Lonza grant Akouos exclusive licenses to proprietary Ancestral AAV (Anc-AAV) vectors for all hearing and balance disorders.
- Anc80, the lead Anc-AAV in a portfolio of 40,000 novel capsids, is a potent gene therapy vector capable of superior gene expression in the inner ear.

**Boston, Mass. and Houston, Texas – November 30, 2017** – Akouos, a new biotechnology company focused on restoring and preserving hearing, has closed a \$7.5 million seed round led by 5AM Ventures and New Enterprise Associates (NEA), with participation from Partners Innovation Fund. Kush Parmar, managing partner at 5AM Ventures, and Ed Mathers, partner at NEA, join the Akouos board of directors as a result of the investment. Concurrent with the financing, Akouos entered into strategic license agreements with Lonza and Massachusetts Eye and Ear for exclusive rights to the Anc-AAV gene therapy platform for all hearing and balance disorders.

“Our mission is to make healthy hearing available to all,” said Dr. Manny Simons, founder and CEO of Akouos. “To this end, we are building the leading inner ear gene therapy company. Our cornerstone partnership with Massachusetts Eye and Ear, the world’s largest hearing research center, and with Lonza, a global leader in viral gene and cell therapy manufacturing, sets a strong foundation for the important work to be done in this emergent field.”

Anc-AAVs are *in silico*-designed adeno-associated viral vectors (AAVs), first developed in the laboratory of Dr. Luk H. Vandenberghe, Assistant Professor of Ophthalmology at Harvard Medical School, and Director of the Grousbeck Gene Therapy Center at Massachusetts Eye and Ear. Under the terms of the agreements, Akouos obtains an exclusive field license to the Anc-AAV IP estate and broad sublicensing rights.

Building upon an existing Anc-AAV partnership between Massachusetts Eye and Ear and Lonza, the agreement with Akouos is designed to accelerate AAV gene therapy development for hearing and balance disorders by bringing together world class expertise and resources in hearing science research and development, clinical research, and AAV manufacturing.

“Together with these strategic partners and our founding investors at 5AM Ventures, NEA, and Partners Innovation Fund, Akouos is poised to translate scientific breakthroughs into life-changing treatments,” continued Dr. Simons. “We are excited to be working collaboratively

with the hearing science research community, hearing health care providers, and families affected by hearing loss to create a future in which the gifts of hearing are available to all.”

“This strategic licensing deal with Akouos, alongside our valued partner in Massachusetts Eye and Ear, is a key milestone on the path to delivering the next transformative class of gene therapies together.” said Marc Funk, COO, Lonza’s Pharma&Biotech segment. “It upholds our commitment at Lonza, the leader and pioneer in global AAV manufacturing, to support world-class innovation in the gene therapy field and our continuous quest for improving patients’ lives.”

“We are very excited to bring together such great talent to accelerate the progress in treatment of hearing and balance restoration disorders,” said D. Bradley Welling, M.D., Ph.D., Chief of Otolaryngology at Massachusetts Eye and Ear and Massachusetts General Hospital and the Walter Augustus LeCompte Professor and Chair of Otolaryngology at Harvard Medical School and Joan W. Miller, M.D., Chief of Ophthalmology at Massachusetts Eye and Ear and Massachusetts General Hospital, and the David Glendenning Cogan Professor of Ophthalmology and Chair of Ophthalmology at Harvard Medical School. “It is our mission to make a difference in the lives of patients.”

### **About Akouos**

Akouos is a biotechnology company focused on restoring and preserving hearing. Akouos’s scientific founders are world leaders in the fields of neurotology, genetics, inner ear drug delivery, and AAV gene therapy:

- Michael J. McKenna, M.D., is a world-renowned neurotologist with expertise in translational inner ear drug delivery research. He holds the Joseph B. Nadol, Jr. Chair and is the director of the Division of Otology and Neurotology at Massachusetts Eye and Ear, and is a professor of otolaryngology at Harvard Medical School.
- William F. Sewell, Ph.D., is a leading expert in inner ear drug delivery and pharmacokinetics. He is professor of otolaryngology at Massachusetts Eye and Ear and Harvard Medical School.
- Richard H. Smith, M.D., is recognized globally as the leading physician-scientist in the field of hereditary hearing loss. He is director of the Molecular Otolaryngology and Renal Research Laboratories and professor of otolaryngology, pediatrics, and molecular physiology and biophysics at the University of Iowa.
- Luk H. Vandenberghe, Ph.D., is distinguished for his breakthroughs in the AAV field, including the discovery and characterization of new AAV serotypes, and improved understanding of immune responses to AAV vectors. He is director of the Grousbeck Gene Therapy Center at Massachusetts Eye and Ear and assistant professor of ophthalmology at Harvard Medical School.

Akouos launched in 2017 with funding from 5AM Ventures, NEA, and Partners Innovation Fund. Further information can be found at [www.akouos.com](http://www.akouos.com).

*Disclosure: Drs. McKenna, Sewell, Smith, and Vandenberghe hold equity and serve on the Scientific Advisory Board of Akouos. In addition, Dr. Vandenberghe is a listed inventor on Anc-AAV intellectual property licensed to Lonza and Akouos for which he receives royalties.*

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### **About Lonza**

Following the closing of the Capsugel acquisition, Lonza further strengthened its position as one of the world's leading and most-trusted suppliers to the pharmaceutical, biotech and specialty ingredients markets. Lonza harnesses science and technology to create products that support safer and healthier living and that enhance the overall quality of life.

An integrated solutions provider serving the healthcare continuum, Lonza offers products and services from the custom development and manufacturing of active pharmaceutical ingredients to innovative dosage forms for the pharma and consumer health and nutrition industries. In addition to drinking water sanitizers, nutraceuticals, antidandruff agents and other personal care ingredients, the company provides agricultural products, advanced coatings and composites and microbial control solutions that combat dangerous viruses, bacteria and other pathogens.

Founded in 1897 in the Swiss Alps, Lonza today is a well-respected global company with more than 50 major manufacturing and R&D facilities and nearly 14,000 full-time employees worldwide. Further information can be found at [www.lonza.com](http://www.lonza.com).

### **Additional Information and Disclaimer**

Lonza Group Ltd has its headquarters in Basel, Switzerland, and is listed on the SIX Swiss Exchange. It has a secondary listing on the Singapore Exchange Securities Trading Limited ("SGX-ST"). Lonza Group Ltd is not subject to the SGX-ST's continuing listing requirements but remains subject to Rules 217 and 751 of the SGX-ST Listing Manual.

Certain matters discussed in this news release may constitute forward-looking statements. These statements are based on current expectations and estimates of Lonza Group Ltd, although Lonza Group Ltd can give no assurance that these expectations and estimates will be achieved. Investors are cautioned that all forward-looking statements involve risks and uncertainty and are qualified in their entirety. The actual results may differ materially in the future from the forward-looking statements included in this news release due to various factors. Furthermore, except as otherwise required by law, Lonza Group Ltd disclaims any intention or obligation to update the statements contained in this news release

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### **About Massachusetts Eye and Ear**

Mass. Eye and Ear clinicians and scientists are driven by a mission to find cures for blindness, deafness, and diseases of the head and neck. Now united with Schepens Eye Research Institute, Mass. Eye and Ear is the world's largest vision and hearing research center, developing new treatments and cures through discovery and innovation. Mass. Eye and Ear is a Harvard Medical School teaching hospital and trains future medical leaders in ophthalmology and otolaryngology, through residency as well as clinical and research fellowships. Internationally acclaimed since its founding in 1824, Mass. Eye and Ear employs full-time, board-certified physicians who offer high-quality and affordable specialty care that ranges from the routine to the very complex. In the 2017-2018 "Best Hospitals Survey," *U.S. News & World Report* ranked Mass. Eye and Ear #2 in the nation for ear, nose, and throat and #4 for eye care. For more information about life-changing care and research, or to learn how you can help, please visit [www.masseyeandear.org](http://www.masseyeandear.org).

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### **About AAV and Anc-AAV technology**

Adeno-associated virus (AAV) vectors have gained traction in gene therapy due to their lack of pathogenicity and the predictability of gene transfer. In the clinic, these vectors have shown the ability to offer gene transfer for treatments of a wide range of diseases including spinal muscular atrophy, hemophilia, and inherited forms of blindness. Anc-AAV technology, developed in the Vandenberghe laboratory, uses computational and evolutionary methods to predict novel conformations of the adeno-associated viral particle. Anc-AAVs share several of the features of naturally occurring AAVs and their variants. However, Anc-AAVs are fully man-made and do not occur in nature. Evidenced by Anc80, this unique synthetic biology and

computational design approach allows for immediate use in therapeutic programs. Furthermore, it provides unprecedented engineering opportunities to overcome deficiencies of current technologies to address diseases of unmet need for broader patient populations. Anc80, a putative ancestor of AAV1, 2, 3, 6, 7, 8, rh.10, and AAV9, has been shown in mice and non-human primates to be a safe and potent therapeutic gene delivery vector in preclinical studies.