AGTC and Otonomy Announce Strategic Collaboration to Develop and Commercialize Gene Therapy for Congenital Hearing Loss

Initial focus on GJB2, the most common cause of genetic hearing loss in newborns

GAINESVILLE, FL, CAMBRIDGE, MA, and SAN DIEGO, CA, October 1, 2019 -- Applied Genetic Technologies Corporation (Nasdaq: AGTC), a biotechnology company conducting human clinical trials of adeno-associated virus (AAV)-based gene therapies for the treatment of rare diseases, and Otonomy, Inc. (NASDAQ: OTIC), a biopharmaceutical company dedicated to the development of innovative therapeutics for neurotology, today announced that they have entered into a strategic collaboration to co-develop and co-commercialize an AAV-based gene therapy to restore hearing in patients with sensorineural hearing loss caused by a mutation in the gap junction protein beta 2 gene (GJB2)— the most common cause of congenital hearing loss.

Mutations in GJB2 account for approximately 30% of all genetic hearing loss cases. Patients with this mutation can have severe-to-profound deafness in both ears that is identified in screening tests routinely performed in newborns. Under the collaboration agreement, the parties will equally share the program costs and proceeds, and can include additional genetic hearing loss targets in the future.

“There is a tremendous unmet need for effective drug therapies to treat sensorineural hearing loss and this is especially true in children born with severe or progressive hearing loss due to genetic mutations,” said Lawrence R. Lustig, M.D., Chair, Department of Otolaryngology – Head & Neck Surgery, Columbia University College of Physicians and Surgeons. “Gene therapy holds great promise to restore functional and lasting hearing in these cases, with early intervention providing significant benefits in the development of language skills, socialization and overall quality of life for patients and their families.”

“We are excited to initiate this collaboration which combines our deep capabilities, technology and expertise in gene therapy development and manufacturing with Otonomy’s extensive experience in otic drug delivery and existing infrastructure for preclinical and clinical development in neurotology,” said Sue Washer, president and CEO of AGTC. “In addition to the positive data that we recently released supporting our XLRP and achromatopsia clinical trials, advancing this program in neurotology further demonstrates the versatility of our gene therapy platform and its applicability to treat a broad range of indications.”

“As leaders in neurotology, we are pleased to add our expertise to that of the highly experienced AGTC team, leveraging what each of us does best and sharing the cost and effort to bring a novel therapeutic to patients with profound need,” said David A. Weber, Ph.D., president and CEO of Otonomy. “The addition of this gene therapy program further extends our broad, innovative pipeline across the largest market opportunities in neurotology including acquired and genetic forms of hearing loss, tinnitus, and balance disorders such as Ménière’s disease.”

About Genetic Hearing Loss

Hearing loss is the most common inherited sensory disorder. In developed countries such as the United States, genetic mutations are responsible for the vast majority of hearing loss in young children, which is estimated to affect 1 out of 500 children before speech develops. Because congenital hearing loss is one

of the most prevalent chronic conditions in children, neonatal screening is routinely performed. This is typically followed by genetic testing in those cases where a deficit has been detected. Mutations have been identified in over 100 genes including the gap junction protein beta 2 gene (GBJ2), which is the most frequent cause of severe-to-profound congenital hearing loss. The GJB2 gene encodes connexin-26 which is expressed in cochlear support cells, forming gap junctions that control potassium homeostasis which is critical for the survival and function of hair cells and normal hearing. Mutations in GJB2 impair gap junctions and cochlear homeostasis leading to hair cell dysfunction and hearing loss. The goal of GJB2 gene therapy is to restore functional gap junctions and preserve hair cells to improve hearing.

About AGTC

AGTC is a clinical-stage biotechnology company that uses a proprietary gene therapy platform to develop transformational genetic therapies for patients suffering from rare and debilitating diseases. Its initial focus is in the field of ophthalmology, where it has active clinical trials in X-linked retinitis pigmentosa (XLRP) and achromatopsia (ACHM CNGB3 & ACHM CNGA3). In addition to its clinical trials, AGTC has preclinical programs in optogenetics, adrenoleukodystrophy (ALD), which is a disease of the central nervous system (CNS) and other ophthalmology and otology indications. The optogenetics program is being developed in collaboration with Bionic Sight. AGTC has a significant intellectual property portfolio and extensive expertise in the design of gene therapy products including capsids, promoters and expression cassettes, as well as expertise in the formulation, manufacture and physical delivery of gene therapy products. For additional information please visit www.agtc.com

About Otonomy

Otonomy is a biopharmaceutical company dedicated to the development of innovative therapeutics for neurotology. The company pioneered the application of drug delivery technology to the ear in order to develop products that achieve sustained drug exposure from a single local administration. This approach is covered by a broad patent estate and is being utilized to develop a pipeline of products addressing important unmet medical needs including Ménière's disease, hearing loss, and tinnitus. For additional information please visit www.otonomy.com.

Cautionary Note Regarding Forward Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements generally relate to future events or the future financial or operating performance of AGTC and/or Otonomy. Forward-looking statements in this press release include, but are not limited to, the potential benefits of and activity under the collaboration agreement between AGTC and Otonomy, including but not limited to development and commercialization activity, and cost and proceeds sharing, the potential benefits of the therapy, the strategy and development activities of AGTC and Otonomy, and statements by the president and CEO of AGTC and the president and CEO of Otonomy. AGTC's and Otonomy's expectations regarding these matters may not materialize, and actual results in future periods are subject to risks and uncertainties. Actual results may differ materially from those indicated by these forward-looking statements as a result of these risks and uncertainties, including but not limited to: the risk of the occurrence of any event, change or other circumstance that could give rise to the termination of the collaboration agreement between AGTC and Otonomy; uncertainties inherent in the clinical drug development process;

competition in the biopharmaceutical industry; expectations regarding either company's expertise and capabilities; AGTC's and Otonomy's ability to protect its intellectual property; expectations regarding potential therapy benefits, market size and opportunity; the ability to obtain regulatory approval for such therapies; AGTC's and Otonomy's ability to manage operating expenses; implementation of their respective business models and strategic plans for their business, products and technology; and other risks. Information regarding the foregoing and additional risks may be found in the section entitled "Risk Factors" in AGTC's Annual Report on Form 10-K for the fiscal year ended June 30, 2019, filed with the Securities and Exchange Commission (the "SEC") and in Otonomy's Quarterly Report on Form 10-Q filed with the SEC on August 1, 2019, and AGTC's and Otonomy's future reports to be filed with the SEC. The forward-looking statements in this press release are based on information available to AGTC and Otonomy as of the date hereof. AGTC and Otonomy each disclaim any obligation to update any forward-looking statements, except as required by law.

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