Otonomy and AGTC Present Preclinical Proof-of-Concept Results for OTO-825 Gene Therapy at ASGCT Annual Meeting

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OTO-825 administration rescues hearing and cochlear damage in two preclinical models of congenital hearing loss caused by GJB2 deficiency

SAN DIEGO, May 14, 2021 (GLOBE NEWSWIRE) -- Otonomy, Inc. (Nasdaq: OTIC), a biopharmaceutical company dedicated to the development of innovative therapeutics for neurotology, today announced preclinical proof-of-concept results for OTO-825 presented at the American Society of Gene & Cell Therapy (ASGCT) Annual Meeting. OTO-825 is an AAV-mediated gene therapy targeting the gap junction beta-2 (GJB2) gene developed under the company's collaboration with Applied Genetic Technologies Corporation (Nasdaq: AGTC). These results demonstrate that a single administration of OTO-825 rescues hearing loss and cochlear damage in two preclinical models that represent a range of hearing loss severity caused by GJB2 deficiency.

"The treatment of GJB2 deficiency is an important unmet medical need since it is the most common genetic mutation causing congenital hearing loss with many patients experiencing significant impairment beginning at birth," said Alan C. Foster, Ph.D., chief scientific officer of Otonomy. "These proof-of-concept results in two independent preclinical models are an important milestone for the program because they validate the therapeutic potential of OTO-825 across a range of hearing loss levels observed in patients and support its advancement into IND enabling activities, which are currently underway."

The joint oral presentation by Otonomy and AGTC demonstrates the following:

- OTO-825 induces expression of Connexin26, the protein product of the GJB2 gene, in target cochlear cells including support cells of the organ of Corti and spiral limbus, and fibrocytes of the spiral ligament in both rodents and non-human primates.
- Connexin26 expressed by OTO-825 forms functional gap junctions, which are required for proper functioning of cochlear hair cells.
- Two conditional knockout models developed in mice mimic the human setting for GJB2 deficiency by displaying hearing loss and damage to the integrity of cochlear tissue.
- A single intracochlear administration of OTO-825 rescues expression of Connexin26 in both models.
- OTO-825 induces significant improvement in hearing across multiple frequencies and normalizes cochlear morphology in both models.

Mutations in the GJB2 gene account for approximately 30% of congenital hearing loss cases. Patients with GJB2 mutation can have severe-to-profound deafness in both ears that is identified in screening tests routinely performed in newborns.

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 with a focus on 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 Otonomy. Forward-looking statements in this press release include, but are not limited to, statements related to plans and expectations regarding OTO-825; and statements by Otonomy's chief scientific officer.

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: delays and disruption resulting from the COVID-19 pandemic; Otonomy's ability to obtain additional financing; the uncertainties inherent in the clinical drug development process, including, without limitation, Otonomy's ability to adequately demonstrate the safety and efficacy of its product candidates and the nonclinical and clinical results for its product candidates, which may not support further development; the risks of the occurrence of any event, change or other circumstance that could impact the performance under or give rise to the termination of Otonomy's collaboration, co-promotion or license agreements, including its collaboration agreement with AGTC, or that could impact Otonomy's ability to repay or comply with the terms of the loan provided by Oxford Finance LLC; side effects or adverse events associated with Otonomy's product candidates; competition in the biopharmaceutical industry; Otonomy's dependence on third parties to conduct nonclinical studies and clinical trials, and for the manufacture of its product candidates; Otonomy's ability to protect its intellectual property in the United States and throughout the world and to ensure compliance with various laws and regulations in countries in which it conducts clinical trials; expectations regarding potential therapy benefits, market size, opportunity and growth; Otonomy's ability to manage operating expenses; implementation of Otonomy's business model and strategic plans for its business,

products and technology; general economic and market conditions; and other risks. Information regarding the foregoing and additional risks may be found in the section entitled "Risk Factors" in Otonomy's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on May 11, 2021, 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 Otonomy as of the date hereof. Otonomy disclaims any obligation to update any forward-looking statements, except as required by law.

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