



## Otonomy and AGTC Present Preclinical Results Supporting Selection of Product Candidate for GJB2 Gene Therapy Hearing Loss Program

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SAN DIEGO, May 12, 2020 (GLOBE NEWSWIRE) -- Otonomy, Inc. (Nasdaq: OTIC), a biopharmaceutical company dedicated to the development of innovative therapeutics for neurotology, today announced preclinical results from the company's gene therapy collaboration with Applied Genetic Technologies Corporation (Nasdaq: AGTC) that support the selection of a product candidate for development as a treatment for gap junction beta-2 (GJB2) deficiency, the most common cause of congenital hearing loss. These results are being presented during the American Society of Gene & Cell Therapy (ASGCT) 23<sup>rd</sup> Annual Meeting being held virtually May 12-15, 2020.

"Working with AGTC, we have identified novel and proprietary capsids that are capable of transducing support cells of the cochlea, which are the relevant target cells for treating GJB2 deficiency," said David A. Weber, Ph.D., president and chief executive officer of Otonomy. "The selection of a lead capsid and other components for continued development of a GJB2 gene therapy candidate is supported by cochlea explant studies in multiple rodent species as well as *in vivo* studies in non-human primates that are summarized in today's ASGCT presentation."

The joint poster presentation by Otonomy and AGTC demonstrate the following:

- A gene of interest can be expressed in cochlear cells relevant to GJB2 rescue, including support cells of the organ of Corti and spiral limbus, and fibrocytes of the spiral ligament.
- The tropism and gene expression level in support cells is higher for these novel capsids than for previously reported capsids used in the otic field.
- Consistent gene expression can be observed in support cells throughout the cochlea for at least 12 weeks following a single local injection.
- None of the novel AAV capsids evaluated for further development exhibited signs of cellular toxicity.

In October 2019, Otonomy and AGTC announced 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 GJB2 gene.

### 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](http://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 expectations regarding the potential benefits, development activity and advancement of preclinical programs; the potential benefits of and activity under the collaboration agreement between AGTC and Otonomy, including but not limited to development activity; and statements by Otonomy's president and CEO. 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 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; Otonomy's ability to obtain regulatory approval for its product candidates; the risks 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; competition in the biopharmaceutical industry; Otonomy's dependence on third parties to conduct nonclinical studies and clinical trials; Otonomy's dependence on third parties for the manufacture of its product candidates; Otonomy's ability to protect its intellectual property related to its product candidates in the United States and throughout the world; 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; 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 (the "SEC") on May 7, 2020, 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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