Otonomy Presents Preclinical Results for GJB2 Gene Therapy Collaboration and Cisplatin Otoprotection Program

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SAN DIEGO, Jan. 28, 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) focused initially on treating GJB2 deficiency for congenital hearing loss, and preclinical results demonstrating the therapeutic potential of a class of compounds being evaluated for otoprotection against cisplatin-induced hearing loss (CIHL). These results were presented during the ongoing Association for Research in Otolaryngology (ARO) 43rd Annual MidWinter Meeting being held in San Jose, California.

"Together with our strategic partner, AGTC, we are encouraged by these initial preclinical results that demonstrate our ability to express a gene of interest in the target cells relevant to the treatment of congenital hearing loss due to GJB2 deficiency," said David A. Weber, Ph.D., president and chief executive officer of Otonomy. "Also, the preclinical results presented for our OTO-510 program highlight the therapeutic potential of a novel class of cisplatin-binding molecules for protection against CIHL and the higher potency of these agents versus other molecules currently in clinical development."

Preclinical Results for GJB2 Gene Therapy Collaboration

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 gap junction protein beta 2 gene (GJB2) -- the most common cause of congenital hearing loss. The joint presentation by Otonomy and AGTC at ARO provided initial demonstration that a gene of interest can be expressed in support cells of the cochlea, which are the relevant target cells for treating GJB2 deficiency, using novel and proprietary AAV capsids. Furthermore, these studies identified several capsids with favorable tropism and gene expression level in support cells compared to previously reported capsids used in the field. Importantly, none of the novel AAV capsids evaluated for further development exhibited signs of cellular toxicity.

Preclinical Results for OTO-510 Otoprotection Program

Cisplatin is a potent chemotherapeutic agent that is widely used to treat a variety of cancers in adults and children. Unfortunately, the administration of cisplatin is commonly associated with severe adverse effects including CIHL that is progressive, bilateral and irreversible. At ARO, Otonomy presented preclinical results demonstrating varying degrees of otoprotection against CIHL for several different classes of therapeutic agents. In particular, a novel proprietary class of agents that potently bind to cisplatin demonstrated greater otoprotection than anti-oxidant and anti-apoptotic molecules, and increased potency relative to other cisplatin-binding molecules currently in clinical development. These results highlight the therapeutic potential of Otonomy's novel otoprotectant agents as the basis for the OTO-510 program for CIHL.

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 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: Otonomy's limited operating history and its expectation that it will incur significant losses for the foreseeable future; Otonomy's ability to accurately forecast financial results; Otonomy's ability to obtain additional financing; Otonomy's dependence on the regulatory success and advancement of its product candidates; 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, the nonclinical and clinical results for its product candidates, which may not support further development, and challenges related to patient enrollment in clinical trials; 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; the risks of the occurrence of any event, change or other circumstance 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; Otonomy's ability to successfully commercialize its product candidates, if approved; 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 dependence on a small number of suppliers for raw materials; 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 November 5, 2019, 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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