Audentes Therapeutics and Genethon Announce Agreement to Develop Treatment for Severe Genetic Disease X-Linked Myotubular Myopathy

Promising preclinical data recently published in Science Translational Medicine

SAN FRANCISCO, CA – February 5, 2014 –Audentes Therapeutics, Inc., a biotechnology company dedicated to the development of innovative treatments for rare muscle diseases, and Genethon, a non-profit organization dedicated to the research and development of biotherapies for orphan genetic diseases, announce that they have entered into an agreement to develop AT001 for the treatment of X-Linked Myotubular Myopathy (XLMTM), a rare, inherited disorder characterized by severe muscle weakness and respiratory impairment. AT001 is a novel drug candidate based on adeno-associated virus (AAV) gene therapy technology.

"Our agreement with Genethon is a significant step forward towards the development of a treatment for patients with this serious, rare disease," said Matthew R. Patterson, President and CEO of Audentes. "The combination of Genethon's expertise in the manufacturing and development of gene therapy products and Audentes' world-class orphan drug development team will allow us to rapidly advance this program."

The development of a potential treatment for XLMTM using gene therapy technology was initiated at Genethon in 2009. Studies by Buj-Bello et al in a mouse model of the disease demonstrated that delivery of the deficient gene using an AAV vector system resulted in increased expression of the protein, improvement in muscle architecture, reversal of muscle hypotrophy, improvement in muscle strength, and an improvement in overall survival (Buj-Bello et al, *Human Molecular Genetics*, 2008, Vol. 17: 2132-2143; Buj-Bello et al, *Molecular Therapy*, 2013, 21:sup 1, S14). More recently, collaborators at Genethon, the University of Washington and Harvard University announced promising results from studies of the same treatment approach in a naturally-occurring dog model of XLMTM (Childers et al., *Science Translational Medicine*, 22 January 2014 6:220ra10). These studies demonstrated that treatment with a single dose of AAV carrying the gene deficient in XLMTM resulted in an increase in muscle strength, improved respiratory function, and prolonged survival. These data are the first demonstration of persistent disease correction in a large animal model of a neuromuscular disease through the delivery of a single, intravenous administration of AAV.

"We are proud of our initial research on this important potential treatment for XLMTM and are very encouraged by the recently published data," explained Frédéric Revah, Chief Executive Officer of Genethon. "We are confident that this collaboration with the experienced team at Audentes will help us achieve our goal of bringing a treatment to patients as soon as possible."

About X-Linked Myotubular Myopathy (XLMTM)
X-Linked Myotubular Myopathy (XLMTM) is a rare, inherited disorder characterized by severe muscle weakness and respiratory impairment. It is caused by mutations in the *MTM1* gene, which

encodes an enzyme called myotubularin. Myotubularin is thought to be involved in the development and maintenance of muscle cells. XLMTM affects 1 in 50,000 newborn males worldwide. In a majority of newborns with the disorder, XLMTM causes death in the first years of life. There is currently no effective treatment for this disease.

About Genethon
Genethon, located in Evry, France, is a non-profit R&D organization dedicated to the development of biotherapeutics for orphan genetic diseases, from research to clinical validation. Genethon is specialized in the discovery and development of gene therapy drugs and has multiple ongoing programs at clinical, preclinical and research stages for neuromuscular, blood, immune system, liver, and eye diseases. To support clinical development of gene therapy drugs, Genethon has built one of the largest facilities worldwide for the production of clinical-grade gene therapy vectors.

For more information regarding Genethon, please visit www.genethon.fr.

About Audentes Therapeutics
Audentes™ is a private development stage biotechnology company committed to the development and commercialization of innovative new treatments for people with serious, rare muscle diseases through the application of adeno-associated virus (AAV) gene therapy technology. The company consists of a focused, experienced, and passionate team driven by the goal of improving lives of patients. Audentes takes pride in strong, global relationships with the patient, research, and medical communities.

For more information regarding Audentes, please visit www.audentestx.com.

Contact
Audentes Therapeutics, Inc.
Jeffrey Gruis
jgruis@audentestx.com
415-638-6561

Genethon

Gaëlle Monfort / Ellia Foucard-Tiab - + 33 1 69 47 28 59 / 25 64 - presse@afm.genethon.fr