



Audentes Therapeutics Announces Six Abstracts to be Presented at the American Society of Gene and Cell Therapy Meeting May 21-24, 2014

Data to be presented demonstrate continued encouraging results from preclinical studies of AT001 for the treatment of X-Linked Myotubular Myopathy

SAN FRANCISCO – May 19, 2014 – Audentes Therapeutics, Inc., a biotechnology company committed to the development and commercialization of gene therapy products for patients with serious, rare diseases, today announced the presentation of results from ongoing preclinical studies of AT001 (AAV-MTM1), Audentes' novel therapeutic in development for the treatment of X-linked Myotubular Myopathy (XLMTM). XLMTM is a rare, inherited disorder characterized by severe muscle weakness and respiratory impairment. Data will be presented at the 17th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT), to be held in Washington D.C. May 21-24.

Previously published data from studies of AT001 in two animal models of XLMTM demonstrate promising proof of concept for the potential of AT001 as a future treatment option for patients with the disease (Buj-Bello et al, *Human Molecular Genetics*, 2008, Vol. 17: 2132-2143; Buj-Bello et al, *Molecular Therapy*, 2013, 21:sup 1, S14; and Childers et al., *Science Translational Medicine*, 22 January 2014 6:220ra10). These animal 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. New data from studies in the naturally occurring dog model of XLMTM, which will be presented at this year's ASGCT, expand and confirm earlier observations. 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. The studies of AT001 have been performed in collaboration with Genethon and UW Medicine.

A list of abstracts is below.

No.	Title	Schedule
402	Goddard <i>et al.</i> , Gait Function in a Canine Model of XLMTM After Systemic Delivery of AAV8-MTM1.	Poster Session: Musculoskeletal Cell and Gene Therapy Thursday 5/22
403	Goddard <i>et al.</i> , Improved Respiratory Function Is Associated with Increased Survival After Systemic Delivery of AAV8-MTM1 in a Canine Model of XLMTM.	Poster Session: Musculoskeletal Cell and Gene Therapy Thursday 5/22
405	Czerniecki <i>et al.</i> Hindlimb, Forelimb Strength Assessment in a Canine Model of XLMTM Following AAV-Mediated Gene Replacement.	Poster Session: Musculoskeletal Cell and Gene Therapy Thursday 5/22
414	Snyder <i>et al.</i> , A Novel Clinical Severity Scoring Measure in Canine XLMTM: Results Following Systemic AAV Gene Replacement.	Poster Session: Musculoskeletal Cell and Gene Therapy Thursday 5/22
513	Mack <i>et al.</i> , Dose-Response Effects Following Systemic Infusion of AAV8-MTM1 on Muscle Strength, Walking Speed, Respiration, Neuromuscular Function and Survival in a Canine Model of XLMTM.	Oral Abstract Session: Therapies for Myogenic Disorders Friday 5/23
764	Buscara <i>et al.</i> , Gene Replacement Therapy of Myotubular Myopathy: Restricting Expression of MTM1 to Skeletal Muscle	Oral Abstract Sessions: Musculoskeletal Cell and Gene Therapy Saturday 5/24

All abstracts for the ASGCT meeting are available online at [2014 ASGCT Annual Meeting Abstracts](#).

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 approximately 1 in 50,000 newborn males worldwide.

About AT001

AT001 is a novel drug candidate for the treatment of XLMTM based on adeno-associated virus (AAV) gene therapy technology. It comprises an AAV containing a full-length MTM1 cDNA (AAV-MTM1). Available data from animal studies suggest that gene therapy using AAV can lead to long-term gene expression, which may translate to a long-term treatment effect for patients. Audentes is developing AT001 in collaboration with Genethon.

About Audentes Therapeutics, Inc.

Audentes is a biotechnology company committed to the development and commercialization of gene therapy products for patients with serious, rare diseases. The company consists of a focused, experienced, and passionate team driven by the goal of improving the 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.

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 specializes 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.

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