



Audentes Therapeutics Announces New Data to be Presented at American Society of Cell and Gene Therapy Annual Meeting

Compelling additional preclinical data further support therapeutic potential of gene therapy products to treat X-Linked Myotubular Myopathy and Pompe Disease

SAN FRANCISCO, CA - May 11, 2015 – Audentes Therapeutics, Inc., a biotechnology company committed to the development and commercialization of gene therapy products for patients with serious, rare diseases, today announced that new data from its lead development programs will be presented at the 18th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT), to be held in New Orleans, Louisiana on May 13-16th, 2015. New findings from recently completed preclinical studies of AT001, Audentes' novel therapeutic in development for the treatment of X-linked Myotubular Myopathy (XLMTM), and AT002, Audentes' novel therapeutic in development for the treatment of Pompe Disease, provide compelling additional proof-of-concept data supporting the rapid advancement of both programs into clinical trials.

AT001 (AAV-MTM1) for XLMTM

- Data from a new, recently completed study in the naturally occurring dog model of XLMTM confirm and expand on earlier observations demonstrating the effects of AAV-MTM1 treatment, including prolonged survival and correction of severe muscle pathology. The data also provide new insights regarding the dose response relationship and biodistribution of AAV-MTM1 in the canine model. These data will be presented by David L. Mack, PhD of the University of Washington School of Medicine (UW Medicine).
- Ana Buj-Bello, MD, PhD of Genethon will present a review of the findings to date evaluating AAV-MTM1 systemic therapy in mouse and dog models of XLMTM. Also, Dr. Buj-Bello will be presented with an Outstanding New Investigator award by the ASGCT for her work towards the development of gene therapy treatment for XLMTM. Audentes congratulates Dr. Buj-Bello on this recognition.

AT002 (AAV-GAA) for Pompe Disease

- Data from a recently completed study comparing the effects of treatment with AAV-GAA gene therapy and treatment with enzyme replacement therapy (ERT) on measures of respiratory, neuromuscular and cardiac function in a mouse model of Pompe disease will be shared in a poster session. The study compared the effects of a single systemic injection of AT002 (AAV-GAA) to bi-monthly injections of recombinant human GAA (ERT) over a three month period. Improvements in diaphragmatic contractile and cardiac function were observed in both treatment groups at three months post-injection compared with untreated animals, while breathing frequency and expiratory time were significantly improved in AAV-GAA treated animals but not in ERT treated animals. In addition, glycogen deposition was significantly elevated in untreated and ERT animals, but not in AAV-GAA treated animals at three months

post treatment. These data highlight not only the therapeutic potential of AAV-GAA treatment in Pompe disease, but also important potential differences in the effects of AAV-GAA compared with ERT. These data will be presented by Darin J. Falk, PhD of the University of Florida, College of Medicine. Full results of this study have recently been published in Falk, et. al., *Molecular Therapy - Methods and Clinical Development* (2015) 2, 15007; doi: 10.1038/mtm.2015.7.

- New insights on the effects of AAV-GAA gene therapy on early, mid and severe neuromuscular pathology in the Gaa $-/-$ mouse model of Pompe Disease will be presented in a poster session by Adrian G. Todd, PhD of the University of Florida, Gainesville. These findings build on recent pre-clinical research showing the adverse impacts of glycogen accumulation on the neuromuscular junction and neuronal and muscle function. The poster presentation will share new data demonstrating that early restoration of GAA activity through systemic AAV-GAA treatment is required to fully restore muscle and nerve function in the Gaa $-/-$ mouse model of Pompe disease. Full data from this study have recently been accepted for publication by the *Annals of Neurology* (doi: 10.1002/ana.24433).

In addition, John T. Gray, PhD, Audentes Vice President of Research and Development, will be speaking about recent advances in the manufacturing of gene therapy products. Using the development of a Hemophilia B gene therapy vector as the therapeutic example, Dr. Gray will summarize recent efforts to define measurable biochemical parameters of viral vector particles that determine drug product potency and safety. Dr. Gray will give his presentation during the Scientific Symposium entitled *Advances in Physical Gene Delivery from Basic Understanding to Translation*, which will be held from 8:00-10:00 am on Friday May 15th, in the Empire C room.

Matthew R. Patterson, President and Chief Executive Officer of Audentes, will be participating in the ASGCT Pre-Conference Commercialization Workshop as a part of the session entitled "C-Suite Executive Panel: Gap analysis and strategic tools for successful commercialization." The panel will be held from 9:35-10:45 am on May 12th, 2015 in the Empire C room.

The abstracts are listed below.

AT001

503

Mack, et. al., Minimally Effective Dose of Systemic AAV8-MTM1 Needed to Prolong Survival and Correct Severe Muscle Pathology in a Canine Model of X-Linked Myotubular Myopathy.

Oral Abstract Session: Musculo-Skeletal Diseases

Date/Time: Friday May 15th, 3:30pm-5:30pm

Room: Celestin F

The abstract for this presentation can be found online at:

http://www.abstracts2view.com/asgct/view.php?nu=ASGCT15L1_503.

Dr. Buj-Bello will give her presentation, entitled “Towards a Gene Therapy for Myotubular Myopathy”, during the Outstanding New Investigator Symposium from 1:15pm to 3:15pm on Thursday, May 14th in Celestin A-E.

AT002

401

Falk, et. al., AAV Therapy Attenuates Respiratory Dysfunction and Glycogen Accumulation in a Murine Model of Glycogen Storage Disease Type II

Poster Session: Musculo-Skeletal Diseases I
Date/Time: Thursday May 14, 2015, 5:30pm–7:00pm
Room: Elite Hall A

The abstract for this poster can be found online at:
http://www.abstracts2view.com/asgct/view.php?nu=ASGCT15L1_401

399

Todd, et. al., Early Correction of Neuromuscular Deficits in Pompe Disease is Required for Restoration of Strength Following Gene Therapy

Poster Session: Musculo-Skeletal Diseases I
Date/Time: Thursday May 14, 2015, 5:30pm-7:00pm
Room: Elite Hall A

The abstract for this poster can be found online at:
http://www.abstracts2view.com/asgct/view.php?nu=ASGCT15L1_399

About X-Linked Myotubular Myopathy (XLMTM)

X-Linked Myotubular Myopathy (XLMTM) is a rare, inherited disorder characterized by severe muscle weakness, respiratory impairment and early mortality. There is no available treatment for the condition. It is caused by mutations in the *MTM1* gene, which encodes an enzyme called myotubularin. Myotubularin plays an important role in the development, maintenance and function of muscle cells. XLMTM affects approximately 1 in 50,000 newborn males worldwide. Audentes is developing AT001 for the treatment of XLMTM in collaboration with Genethon (www.genethon.fr). AT001 is a novel product based on AAV gene therapy technology.

About Pompe Disease

Pompe Disease is a rare, inherited disorder characterized by progressive muscle weakness and respiratory impairment. It is caused by mutations in a gene that encodes an enzyme called acid alpha-glucosidase (GAA), which is needed by the body to break down glycogen – a stored form of sugar used for energy. Pompe Disease affects approximately 1 in every 40,000 births. AT002 is a novel product based on AAV gene therapy technology.

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 has two products in development, AT001 for the treatment of X-Linked Myotubular Myopathy (XLMTM) and AT002 for the treatment of Pompe Disease. 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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