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Audentes Therapeutics Announces Dosing of First Patient in ASPIRO, a Phase 1/2 Clinical Trial of AT132 for the Treatment of X-Linked Myotubular Myopathy

Preliminary data from ASPIRO expected to be available in the fourth quarter of 2017

SAN FRANCISCO, September 21, 2017 / PRNewswire/ -- Audentes Therapeutics, Inc. (Nasdaq: BOLD), a biotechnology company focused on developing and commercializing gene therapy products for patients living with serious, life-threatening rare diseases, today announced it has commenced dosing of patients in ASPIRO, a Phase 1/2 clinical trial of AT132 for the treatment of X-Linked Myotubular Myopathy (XLMTM). ASPIRO is a multicenter, multinational, open-label, ascending dose study to evaluate the safety and preliminary efficacy of AT132 in approximately twelve XLMTM patients less than five years of age. Preliminary data from ASPIRO is expected to be available in the fourth quarter of 2017.

"XLMTM is a severe rare disease characterized by profound muscle weakness, respiratory failure and early death," stated Suyash Prasad, M.D., Senior Vice President and Chief Medical Officer. "Over 50 percent of the boys affected by XLMTM do not live to celebrate their second birthday, and no currently approved treatment options exist for these patients, their families and care-givers."

Dr. Prasad continued, "We are grateful to the many expert collaborators, patient advocacy organizations and parents who have supported Audentes and helped guide our efforts. Most of all we are thankful to the children and families affected by XLMTM for their participation in ASPIRO. We look forward to working together with our partners to advance AT132 as a potentially transformative therapy to treat this devastating disease."

In addition to ASPIRO, the clinical development program for AT132 includes RECENSUS, a retrospective medical chart review, for which Audentes has previously announced data from an initial analysis of 112 male subjects. This analysis confirmed and expanded upon the understanding of the significant disease burden of XLMTM on patients, families and the healthcare system. Audentes is also conducting INCEPTUS, a prospective natural history run-in study. The primary objectives of INCEPTUS are to characterize the clinical condition of children with XLMTM, identify subjects for potential enrollment in ASPIRO, and serve as a longitundinal baseline and within-patient control for ASPIRO. Audentes plans to announce preliminary data from INCEPTUS in the third quarter of 2017.

About AT132 for X-Linked Myotubular Myopathy

AT132 is the Audentes product candidate being developed to treat XLMTM, a rare monogenic disease characterized by extreme muscle weakness, respiratory failure and early death, with an estimated 50% mortality rate by 18 months of age. XLMTM is caused by mutations in the MTM1 gene, which encodes a protein called myotubularin. Myotubularin plays an important role in the development, maintenance and function of skeletal muscle cells. AT132 is comprised of an AAV8 vector containing a functional copy of the MTM1 gene. Multiple studies in animal models of XLMTM have demonstrated that a single administration of AT132 improved disease symptoms and survival rates, with no significant AT132-related adverse events or safety findings. In one study these effects have lasted more than four and a half years years. Audentes is developing AT132 in collaboration with Genethon (www.genethon.fr).

About ASPIRO, the Phase 1/2 Clinical Study of AT132
ASPIRO is designed as a multicenter, multinational, open-label, ascending dose study to evaluate the safety and preliminary efficacy of AT132 in approximately 12 XLMTM patients less than five years of age. The study is expected to include nine AT132 treated subjects and three delayed-treatment concurrent control subjects. Primary endpoints include safety (adverse events and certain laboratory measures) and efficacy (assessments of neuromuscular and respiratory function). Secondary endpoints include the burden of disease and health related quality-of-life, and muscle tissue histology and biomarkers. The primary efficacy analysis is expected to be conducted at 12 months, with interim evaluations expected to be conducted at earlier time points. After the primary 12-month assessment, subjects are expected to be followed for another four years to assess long term safety, durability of effect and developmental progression.

About Audentes Therapeutics, Inc.
Audentes Therapeutics (Nasdaq: BOLD) is a biotechnology company focused on developing and commercializing gene therapy products for patients living with serious, life-threatening rare diseases. We have four product candidates in development, AT132 for the treatment of X-Linked Myotubular Myopathy (XLMTM), AT342 for the treatment of Crigler-Najjar Syndrome, AT982 for the treatment of Pompe disease, and AT307 for the treatment of the CASQ2 subtype of Catecholaminergic Polymorphic Ventricular Tachycardia (CASQ2-CPVT). We are a focused, experienced and passionate team committed to forging 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 biotherapies 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 stage for neuromuscular, blood, immune system, liver and eye diseases.

Discover Genethon's pipeline: http://www.genethon.fr/produits/

Forward Looking Statements
This press release contains forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995, including, but not limited to: the timing of preliminary data from ASPIRO, the timing of data presentation from INCEPTUS and the ability of INCEPTUS to serve as a longitudinal baseline and within patient control for ASPIRO, and the potential of AT132 to be a transformative therapy for patients living with XLMTM. All statements other than statements of historical fact are statements that could be deemed forward-looking statements. Although the company believes that the expectations reflected in such forward-looking statements are reasonable, the company cannot guarantee future events, results, actions, levels of activity, performance or achievements, and the timing and results of biotechnology development and potential regulatory approval is inherently uncertain. Forward-looking statements are subject to risks and uncertainties that may cause the company's actual activities or results to differ significantly from those expressed in any forward-looking statement, including risks and uncertainties related to the company's ability to advance its product candidates, obtain regulatory approval of and ultimately commercial its product candidates, the timing and results of preclinical and clinical trials, the company's ability to fund development activities and achieve development goals, the company's ability to protect

intellectual property and other risks and uncertainties described under the heading "Risk Factors" in documents the company files from time to time with the Securities and Exchange Commission. These forward-looking statements speak only as of the date of this press release, and the company undertakes no obligation to revise or update any forward-looking statements to reflect events or circumstances after the date hereof.

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