



Audentes Therapeutics Announces Presentation Of Data From RECENSUS, A Medical Chart Review Of Patients With X-Linked Myotubular Myopathy (XLMTM)

Data confirm and expand understanding of significant medical burden associated with XLMTM

San Francisco, Calif., March 20, 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 that data from RECENSUS, a medical chart review of patients with X-linked Myotubular Myopathy (XLMTM), will be presented at the 2017 Muscular Dystrophy Association (MDA) Scientific Conference, which will be held in Arlington, Virginia from March 19 to 22, 2017. These data provide new insights into the significant medical burden for children with XLMTM, their families and caregivers.

“A Multicenter, Retrospective Medical Record Review of Patients with X-Linked Myotubular Myopathy (XLMTM): The RECENSUS Study” will be presented by Alan Beggs, PhD, Director of The Manton Center for Orphan Disease Research at Boston Children's Hospital, Sir Edwin and Lady Manton Professor of Pediatrics at Harvard Medical School, and Principal Investigator of the RECENSUS study.

This initial analysis of 112 male patients is the first publication to describe the substantial humanistic and economic burden on the lives of XLMTM children, their families and the healthcare system. Consistent with previous studies, RECENSUS data show that XLMTM is a devastating, life-threatening disease manifesting early in the neonatal period with considerable, ongoing unmet medical need. Key observations include:

- Overall mortality was 44% (64% of patients \leq 18 months of age; 32% of patients $>$ 18 months of age)
- In the first year of life, infants with XLMTM spent 35% of their time in the hospital and underwent an average of 3.7 surgeries
- At birth, 95% of the boys were hypotonic and 90% required respiratory support
- 48% of the boys required 24-hour ventilation and 60% had received a tracheostomy. Those patients that were not ventilated 24-hours per day still spent an average of 8.5 hours daily on a ventilator
- The majority of patients for whom data were available were receiving the most invasive forms of ventilatory support (67% - CPAP/BiPAP, and 64% - IPPV/SIMV/Pressure support)

The data also demonstrate that the time from presentation of symptoms to a confirmed diagnosis of XLMTM is declining, which likely represents an increasing physician awareness of XLMTM, coupled with improved diagnostic techniques. Since the discovery of the *MTM1* gene in 1996, the mean age at diagnosis has dropped from 35.1 months in the period 1996-2000, to 4.4 months in the period 2011-2014. “RECENSUS has established one of the largest data sets of XLMTM in the world, and this analysis makes a vital contribution to our understanding of this terrible disease,” stated Dr. Alan Beggs. “The RECENSUS study more completely defines the disease burden and management of XLMTM, and demonstrates the devastating impact that an XLMTM diagnosis has on the lives of patients and their families.”

While retrospective studies of observational data must be interpreted with caution, they are particularly important in the rare disease setting, where large populations of patients are not available for enrollment in prospective studies. Such studies are critical for exploring the signs, symptoms, management, and burden of rare diseases, and have previously been used to provide historical control populations for new therapies undergoing regulatory approval. In addition, data from the RECENSUS study provide important information to aid in selecting endpoints for interventional studies of XLMTM, including measures of survival, respiratory function, and burden of illness.

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 was well tolerated and significantly improved disease symptoms and survival rates. In one study these effects have lasted more than four years to date.

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.

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 interpretation of the data from RECENSUS and whether it contributes to the overall understanding of the burden of illness and burden of care of XLMTM, whether a study such as RECENSUS can be used to establish a historical control for AT132 as it seeks regulatory approval, and whether RECENSUS will be valuable in establishing or interpreting endpoints in future clinical trials of AT132. 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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