

Audentes Therapeutics Announces FDA Lifts Hold on ASPIRO Clinical Trial of AT132 for Treatment of X-Linked Myotubular Myopathy (XLMTM)

SAN FRANCISCO, CA – Dec 24, 2020 /BUSINESSWIRE/ – [Audentes Therapeutics](#), an Astellas genetic medicines company, today announced that the U.S. Food and Drug Administration (FDA) has lifted the clinical hold for the ASPIRO clinical trial evaluating AT132 in patients with X-linked myotubular myopathy (XLMTM). XLMTM is a serious, life-threatening neuromuscular disease characterized by extreme muscle weakness, respiratory failure, and early death.

“We are grateful for the efforts of our team and investigators who have worked tirelessly to answer the FDA’s questions and we now look forward to resuming this study,” said Natalie Holles, President and Chief Executive Officer of Audentes. “We want to again extend our deepest sympathies to patients’ families impacted by the events earlier this year. We are deeply committed to the continued safe development of AT132 for the families and patients living with XLMTM, a disease with no existing treatments.”

The company is now working to complete all clinical and regulatory activities necessary to resume dosing and plans to have discussions at a future date with the regulators on the path forward toward global registration filings for AT132.

About X-linked Myotubular Myopathy

XLMTM is a serious, life-threatening, rare neuromuscular disease that is characterized by extreme muscle weakness, respiratory failure and early death. Mortality rates are estimated to be 50 percent in the first 18 months of life. For those patients who survive past infancy, there is an estimated additional 25 percent mortality by the age of 10. XLMTM is caused by mutations in the MTM1 gene that lead to a lack or dysfunction of myotubularin, a protein that is needed for normal development, maturation and function of skeletal muscle cells. The disease affects approximately 1 in 40,000 to 50,000 newborn males.

XLMTM places a substantial burden of care on patients, families and the healthcare system, including high rates of healthcare utilization, hospitalization and surgical intervention. More than 80 percent of XLMTM patients require ventilator support, and the majority of patients require a gastrostomy tube for nutritional support. In most patients, normal developmental motor milestones are delayed or never achieved. Currently, only supportive treatment options, such as ventilator use or a feeding tube, are available.

About the ASPIRO Study

ASPIRO is a two-part, multinational, randomized, open-label ascending dose trial to evaluate the safety and preliminary efficacy of AT132 in XLMTM patients less than five years of age. 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.

About AT132 for the treatment of X-linked Myotubular Myopathy

Audentes is developing AT132, an AAV8 vector containing a functional copy of the MTM1 gene, for the treatment of XLMTM. AT132 may provide patients with significantly improved outcomes based on the ability of AAV8 to target skeletal muscle and increase myotubularin expression in targeted tissues following a single intravenous administration. The preclinical development of AT132 was conducted in collaboration with Genethon (www.genethon.fr).

AT132 has been granted Regenerative Medicine and Advanced Therapy (RMAT), Rare Pediatric Disease, Fast Track, and Orphan Drug designations by the U.S. Food and Drug Administration (FDA), and Priority Medicines (PRIME) and Orphan Drug designations by the European Medicines Agency (EMA).

About Audentes Therapeutics, Inc.

Audentes Therapeutics, an Astellas company, is developing genetic medicines with the potential to deliver transformative value for patients. Based on our innovative scientific approach and industry leading internal manufacturing capability and expertise, we have become the Astellas Center of Excellence for the newly created Genetic Regulation Focus Area. We are currently exploring three gene therapy modalities: gene replacement, exon skipping gene therapy, and vectorized RNA knockdown, with plans to expand our focus and geographic reach under Astellas. We are based in San Francisco, with manufacturing and laboratory facilities in South San Francisco and Sanford, North Carolina.

About Astellas

Astellas Pharma Inc. is a pharmaceutical company conducting business in more than 70 countries around the world. We are promoting the Focus Area Approach that is designed to identify opportunities for the continuous creation of new drugs to address diseases with high unmet medical needs by focusing on Biology and Modality. Furthermore, we are also looking beyond our foundational Rx focus to create Rx+® healthcare solutions combine our expertise and knowledge with cutting-edge technology in different fields of external partners. Through these efforts, Astellas stands on the forefront of healthcare change to turn innovative science into value for patients. For more information, please visit our website at <https://www.astellas.com/en>

Cautionary Notes

In this press release, statements made with respect to current plans, estimates, strategies and beliefs and other statements that are not historical facts are forward-looking statements about the future performance of Astellas. These statements are based on management's current assumptions and beliefs in light of the information currently available to it and involve known and unknown risks and uncertainties. A number of factors could cause actual results to differ materially from those discussed in the forward-looking statements. Such factors include, but are not limited to: (i) changes in general economic conditions and in laws and regulations, relating to pharmaceutical markets, (ii) currency exchange rate fluctuations, (iii) delays in new product launches, (iv) the inability of Astellas to market existing and new products effectively, (v) the inability of Astellas to continue to effectively research and develop products accepted by customers in highly competitive markets, and (vi) infringements of Astellas' intellectual property rights by third parties.

Information about pharmaceutical products (including products currently in development) which is included in this press release is not intended to constitute an advertisement or medical advice.



Audentes contacts:

Sarah Spencer

Audentes Therapeutics

Tel: 215-733-9375

sspencer@audentestx.com

Ryan Flinn

Audentes Therapeutics

Tel: 510-207-7616

rflinn@audentestx.com