Audentes Therapeutics Provides Update on the ASPIRO Clinical Trial Evaluating AT132 in Patients with X-linked Myotubular Myopathy

San Francisco, Calif., August 20, 2020 /BUSINESSWIRE/ -- Audentes Therapeutics, Inc., an Astellas company, today announced that a third study patient has passed away in the ASPIRO clinical trial evaluating AT132 in patients with X-linked Myotubular Myopathy (XLMTM), which is a serious, life-threatening neuromuscular disease characterized by extreme muscle weakness, respiratory failure, and early death. Preliminary findings indicate that the immediate cause of death was gastrointestinal bleeding.

This patient was one of three study patients previously disclosed to have received AT132 at the dose of $3x10^{14}$ vg/kg who began to demonstrate signs of liver dysfunction within 3 to 4 weeks after dosing. All three patients demonstrated evidence of pre-existing hepatobiliary disease.

More than 50% of patients enrolled in the ASPIRO trial to date show evidence of pre-existing hepatobiliary disease, but it has not been associated with similar progressive liver dysfunction in any of the patients who received AT132 at the $1x10^{14}$ vg/kg dose nor in the other patients who received the $3x10^{14}$ vg/kg dose.

To date, 23 ASPIRO patients have received AT132: six at the $1x10^{14}$ vg/kg dose and 17 at the $3x10^{14}$ vg/kg dose. Although the ASPIRO study is currently on clinical hold, there are no other patients in the study known to be currently experiencing similar liver dysfunction. Audentes, together with the ASPIRO investigators and independent Data Monitoring Committee, continue to closely monitor all patients enrolled in the study. Additionally, Audentes' investigation regarding why these three patients developed progressive liver dysfunction is ongoing.

Audentes extends its deepest sympathies to this patient's family; the company remains committed to the AT132 development program and the XLMTM patient community. The company plans to provide further information on the ASPIRO program based on both ongoing data collection and future regulatory status updates.

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.

For more information regarding Audentes, please visit www.audentestx.com.

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.

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