AUDENTES THERAPEUTICS

October 16, 2017

Dear XLMTM Community,

As many of you know, we recently dosed the first patient in the clinical trial of AT132 (ASPIRO), the Audentes gene therapy product candidate being developed for the treatment of X-linked Myotubular Myopathy. The purpose of this letter is to let you know how information will be communicated as the ASPIRO clinical trial progresses.

The overall aim of the XLMTM clinical development program is to determine the safety and efficacy of AT132 in the treatment of XLMTM, and bring its potential benefit to children and families living with XLMTM as rapidly as the data support. To achieve these goals, we must run a robustly designed and executed clinical trial which is managed to an exceptionally high standard. Accordingly, findings from the ASPIRO trial will only be shared on a periodic basis. This is because there is a need to appropriately assess and understand the clinical data (which may take some time) before it is released publicly. This assessment period is typical for a clinical trial.

Given the importance of this trial to the XLMTM community, we wanted to inform you of our plans to provide updates on study progress. These updates will take the form of periodic press releases, hosted conference calls and web-casts from investor focused conferences, each of which may be accessed on our web-site. If you are interested in joining our mailing list to automatically receive notifications of these events, we encourage you to sign up on the Investors and Media page at www.audentestx.com. We also plan to provide more detailed reviews of study results at appropriate scientific and medical congresses.

Importantly, we would like to ask for your partnership in helping the XLMTM community understand the need to refrain from any discussion on social media about how the children in ASPIRO may be doing while in the clinical trial. This is critical in helping to maintain the integrity of the information coming out of the trial.

Of course, we realize that there may be specific questions from families about the ASPIRO clinical trial. When participating in a clinical trial, it is critical that such communication only goes through the clinical trial doctor responsible for the study at a particular site, and his/her appointed staff. These medical staff have the ability to answer questions regarding the clinical trial.

Information about the clinical trials may be found online at www.clinicaltrials.gov or at the direct links below:

- INCEPTUS: https://www.clinicaltrials.gov/ct2/show/NCT02704273?term=inceptus&rank=1
- ASPIRO: https://clinicaltrials.gov/ct2/show/NCT03199469?term=aspiro&rank=1

We hope this information is helpful. Please feel free to share this letter with your patient communities.

- If parents of children in the clinical trial have questions, we suggest they directly contact their clinical trial doctor and staff with questions.
- For general inquiries, Audentes Patient Advocacy can be contacted at: patientadvocacy@audentestx.com

We share your urgency in moving this clinical development program forward and remain fully committed to the goal of demonstrating safety and effectiveness of the investigational gene therapy product AT132 as rapidly as possible, for all children and families affected by XLMTM around the world. We hope that we can rely on your collaboration and partnership as we work towards this goal. We look forward to letting you know how things progress.

Sincerely,

Suyash Prasad MD, SVP and Chief Medical Officer

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