

January 4, 2018

Dear XLMTM Patient Leaders,

In 2017, boys affected by X-Linked Myotubular Myopathy were dosed in the ASPIRO clinical trial of AT132, the investigational Audentes gene therapy product candidate being developed for the treatment of XLMTM. The purpose of this letter is to provide you an update on the first interim data from the first dose cohort of ASPIRO.

Because of the considerable interest in these early findings within the XLMTM patient community, we wanted to share this information with you today, as well as a link to the press release that was issued this morning: http://investors.audentestx.com/

It is important to know that this is a <u>preliminary</u> data set—the first of many to be analyzed within this clinical trial—and we will only have more complete information about the potential efficacy or safety of the investigational gene therapy product after all enrolled patients are dosed and the full scope of data is collected from the clinical trial. We would also like to remind everyone that the overall aim of the XLMTM clinical development program is to determine safety and efficacy of AT132 in the treatment of XLMTM and to do it as rapidly as the data supports.

We recognize the importance of this trial to the XLMTM community and will continue to provide updates on progress of the clinical trial. These updates will take the form of periodic press releases, hosted conference calls and webcasts from investor-focused conferences, each of which may be accessed from our website (www.audentestx.com). We also plan to provide more detailed reviews of the study results at appropriate scientific and medical congresses.

We would like to ask for your continued partnership in helping the XLMTM community understand the need to refrain from any discussions (including social media, and other online or offline communications) about how the children in ASPIRO may be doing while the clinical trial is in progress. This includes a sincere request to the XLMTM patient community to please refrain from proactively asking parents of children enrolled in ASPIRO for information regarding their child's medical status during the conduct of the study. This is critical in helping to maintain the integrity of the data coming out of the trial.

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, Kimberly Trant, Director of Audentes Patient Advocacy can be contacted at: patientadvocacy@audentestx.com

We share your urgency in moving this clinical development program forward and thank you for your collaboration and partnership as we work towards this goal.

Sincerely,

Suyash Prasad MD, Pediatrician, Senior Vice President and Chief Medical Officer