AUDENTES THERAPEUTICS

May 1, 2019

Dear Patient Leaders of the XLMTM Community,

In September 2017, the Phase 1/2 ASPIRO clinical trial began to study an investigational gene therapy product candidate in boys affected by X-Linked Myotubular Myopathy (XLMTM). Since that time, we have provided some preliminary findings from this clinical trial to the community. Today, additional information was shared with healthcare providers at a major medical and scientific conference, the 22nd Annual American Society of Gene & Cell Therapy, which was held in Washington, D.C. A press release was also issued today. The press release and the presentation materials can be found in the Investors + Media section of our website at www.audentestx.com.

We are sharing this letter as part of our commitment to ongoing, open communication with patient leaders of the XLMTM patient community. Because of the considerable interest in the early findings from the ASPIRO study, we recognize the need for clarity regarding information as it becomes publicly available. Therefore, we wanted to answer some questions you may have and provide context to the press release and materials issued today.

The information presented today, May 1st, 2019, is based on data that was collected as of April 8, 2019.

What are the goals of the Phase 1/2 ASPIRO investigational gene therapy clinical trial?

- To learn about the safety of the investigational gene therapy product candidate
- To learn whether the investigational gene therapy product candidate is effective for the long-term production of myotubularin, the missing or defective protein in XLMTM
- To determine the appropriate amount, or optimal dose, of the investigational gene therapy product candidate

How many participants have been dosed or randomized to the delayed-treatment control in the Phase 1/2 ASPIRO investigational gene therapy clinical trial?

- Nine (9) participants have been dosed with the investigational gene therapy product candidate
  - Six (6) participants have been given the first dose level in Cohort 1
  - Three (3) participants have been given the second dose level in Cohort 2
- Two (2) participants have been randomized to the delayed-treatment control arm of the clinical trial (one in each cohort), meaning that they will receive the optimal dose (which is yet to be determined) of the investigational gene therapy product candidate later in the clinical trial

What are the early, interim findings in the clinical trial?

Please refer to the press release and presentation materials for the interim findings we reported today.

It is important to note:

- We cannot make any firm conclusions about the findings of the clinical trial until all subjects are dosed and evaluated for the duration of the study, and the full scope of data is collected and analyzed
- Once the study has completed and data has been analyzed, more complete information about the safety and efficacy of this investigational gene therapy product candidate will become available to the community

It is important to understand that regulatory agencies have not approved the Audentes investigational gene therapy product candidate as safe or effective, as it is still undergoing formal assessment in clinical trials. The investigational gene therapy product is not approved for commercial sale and is only available in clinical trial settings.

What are the next steps for the Phase 1/2 ASPIRO investigational gene therapy clinical trial?

- The ASPIRO clinical trial is ongoing with sites activated in the United States, Canada and Europe
- Selection of the optimal dose in the ASPIRO clinical trial is planned to occur in the second quarter of 2019
- Further interactions with the U.S. Food and Drug Administration (FDA) and European Medicines Association (EMA) are planned to occur in the third quarter of 2019
    - Audentes plans to present this most recent data update and to gain further alignment on the license application submission pathways for the investigational gene therapy product candidate in the United States and Europe

When will the next release of findings from the ASPIRO clinical trial take place?

- Audentes typically provides updates at leading scientific conferences. While we do not have an exact date for the next release of information, we do plan to keep the patient and scientific community updated on ASPIRO progress as appropriate through press releases, hosted conference calls and scientific forums.

Is the ASPIRO clinical trial still enrolling?

- Yes, ASPIRO is currently enrolling patients
- Each clinical trial site maintains a wait-list which they manage for patients interested in participating in the clinical trials. These sites continue to evaluate potential patients on these wait-lists based on the protocol criteria and in case there is a future need to enroll more patients into the clinical trials in the case that regulators require this.
- In addition to the inclusion/exclusion criteria listed on clinicaltrials.gov, each clinical trial site may have additional considerations, such as capacity at their site. The overall number of patients in the clinical trial is also limited.

Where can general information about the clinical trial design be found?

- USA: Visit ClinicalTrials.gov and enter the term "ASPIRO"
    - https://clinicaltrials.gov/ct2/show/NCT03199469?term=aspiro&rank=1
- Europe: Visit EU Clinical Trials Register at www.clinicaltrialsregister.eu and enter the term "ASPIRO"
    - https://www.clinicaltrialsregister.eu/ctr-search/trial/2017-000876-27/DE

We would like to ask for your continued partnership in understanding the need to refrain from any discussions (including social media, media, online, telephone or in-person communications) about how children in the ASPIRO clinical trial may be doing while the trial is in progress. This includes friends, families and patient groups. Please refrain from proactively asking parents of children enrolled in the ASPIRO clinical trial for information regarding their child's medical status. This is critical in helping to maintain the integrity of the data coming out of the trial.

Our hope is to demonstrate the safety and efficacy of the investigational gene therapy product candidate to meet the needs of the regulators, such that it will benefit as many children and families affected by XLMTM as possible, in the shortest amount of time. We do this best by running a robustly controlled and scientifically disciplined clinical trial and we need your help in making sure this occurs.

We hope this information is helpful in answering some of the questions you may have.

- If you are a parent or caregiver of a child enrolled in the clinical trial, you should direct all questions to the clinical trial doctor or his/her staff
- For general inquiries, Patient Advocacy at Audentes Therapeutics can be contacted at: patientadvocacy@audentestx.com

We look forward to sharing further information in the near future.

Sincerely,

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