AUDENTES
An Astellas Company

June 23, 2020

Dear XLMTM Patient Community,

A second patient in the ASPIRO clinical trial investigating AT132 (the investigational gene therapy product candidate) for X-linked myotubular myopathy (XLMTM) has tragically passed away. We are deeply saddened by this loss and our hearts go out to the family.

Here is what we can share at this time:

- Preliminary findings indicate that the immediate cause of death was sepsis. This patient had progressive liver dysfunction characterized by hyperbilirubinemia that occurred within the first 4-6 weeks following AT132 dosing, and which did not respond to standard treatment. This patient received aggressive medical treatment for his liver dysfunction, including hospitalization and close clinical observation. Unfortunately his condition worsened, and he ultimately succumbed to bacterial infection and sepsis. Additional information surrounding the death is being collected at this time.
- As was the case with the patient death we shared last month, this patient was also one of three older patients who received AT132 at the dose of $3x10^{14}$ vg/kg in whom we have recently observed new serious adverse events (SAEs) of hepatobiliary disease. Although the investigations around both deaths are ongoing, preliminary reports indicate that the clinical course was similar in the two patients who passed away.
- Notable features among the three patients with these SAEs include older age, heavier weight, evidence of pre-existing hepatobiliary disease, and dosing with the higher dose of $3x10^{14}$ vg/kg. It should be noted that among the six patients treated at $1x10^{14}$ vg/kg, including four with a previous history of hepatobiliary disease, none have developed liver SAEs, despite being years out from treatment.

Next steps:
Our ongoing investigation includes, among other potential factors, an examination of whether the factors described above contributed to these SAEs. The investigation of these cases in the ASPIRO clinical trial continues to be ongoing.

We continue to be in regular contact with the ASPIRO clinical investigators and the ASPIRO Data Monitoring Committee (DMC) as new information becomes available. Regulatory authorities are being notified via their standard reporting procedures.

Prior to both of these deaths, Audentes, in consultation with the DMC, had halted further dosing of patients currently enrolled in the clinical trial. Subsequently, following interactions with the FDA (US Food and Drug Administration), the study was put on formal clinical hold. Per FDA's guidance, we continue to follow and actively monitor those patients who are enrolled in the trial.

We are taking all necessary steps to understand these events and incorporate what we learn into our development plan going forward. We are currently assessing the impact on potential regulatory filing timelines, however we will not be filing in mid-2020 as previously communicated.

We will continue to provide immediate updates on any significant developments related to patient safety in the AT132 development program, regardless of the status of the investigation or clinical trial. We will continue to provide "data cut" updates at appropriate scientific meetings as the progress of ASPIRO and the COVID-19 related delays in data collection allow.

Contact information

- If your child is currently participating in the ASPIRO clinical trial, please speak with the physician and their staff at your clinical trial site if you have questions or would like to discuss the situation.
- If your child is not participating in the clinical trial, and you would like to speak with someone at Audentes, you may contact our Patient Advocacy & Engagement team at: patientadvocacy@audentestx.com

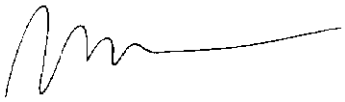
Important note

It is important to understand that regulatory agencies have not approved the Audentes investigational gene therapy product candidate (AT132) or determined that it is safe or effective, as it is still undergoing formal assessment in clinical trials. The investigational gene therapy product candidate is not approved for commercial sale and is only available in clinical trial settings.

We remain committed to developing AT132 for the families and patients living with XLMTM.

Sincerely,

Natalie Holles, President and Chief Executive Officer



Edward Conner, M.D., Senior Vice President and Chief Medical Officer