June 26, 2022

Dear Global Pompe Patient Leaders,

On June 26, 2022, Astellas Pharma, Inc. (“Astellas”) issued a press release announcing that the U.S. Food and Drug Administration (FDA) has placed a clinical hold on the FORTIS Phase 1/2 clinical trial evaluating AT845 (“the investigational gene therapy candidate”) in patients with Late Onset Pompe Disease (LOPD). The hold was placed following the occurrence of a serious adverse event (SAE) of peripheral sensory neuropathy in one of the trial participants.

You can read the entire press release at the following link:
https://www.astellas.com/en/news/25956

Here is what we can share at this time:

- The decision follows the reporting of a serious adverse event (SAE) in one of the study participants due to the development of peripheral sensory neuropathy.
- To date, the SAE has been classified by the site investigator as mild in severity (grade 1) and deemed serious due to medical significance.
- After reporting the SAE to FDA, a clinical hold was then placed on the FORTIS trial.
- The FDA informed Astellas that it did not have sufficient information to assess the risks to subjects and requires additional information about the reported SAE.
- A written explanation for the basis of the hold will be issued by the FDA and sent to Astellas within the next 30 days.
- Astellas is working with the site investigator to closely follow the patient's clinical course and will continue to gather and review all relevant data.
- Close monitoring of all currently enrolled participants will continue per the study protocol.
- There is no relationship between the SAE and the subsequent clinical hold on the FORTIS trial and the ongoing clinical hold for Astellas Gene Therapies’ program for X-linked Myotubular Myopathy (XLMTM).

Additional background:

- The FORTIS clinical trial is designed to evaluate the safety and tolerability of the investigational gene therapy candidate AT845 in adults with LOPD.
- Participants enrolled in FORTIS receive a one-time peripheral intravenous infusion of the investigational gene therapy candidate AT845, followed by one year of frequent monitoring and four additional years of long-term safety monitoring.
- To date, four participants in the FORTIS clinical trial have received the investigational gene therapy candidate.

Next steps:

- Astellas is evaluating the data and all factors that may have contributed to this SAE.
- The Astellas Medical Monitor and FORTIS investigators will continue to monitor the individual and all study participants for all safety outcomes.

Important note:

- The information provided in this letter regarding the investigational gene therapy candidate is being provided for informational and educational purposes only.
- No regulatory agency in any country has approved the investigational gene therapy candidate or determined that it is safe or effective, as it is still undergoing formal assessment in clinical trials.
- There is no guarantee that the investigational gene therapy candidate will receive regulatory approval or become commercially available for the uses being investigated in any country.

Contact information to support with questions:

- Individuals currently participating in the FORTIS clinical trial should speak with the physician and the staff at their clinical trial site if they have questions or would like to discuss the situation.
- Patient Leaders who would like to speak with someone at Astellas Gene Therapies may contact our Patient Advocacy & Engagement team at: AGT_patientadvocacy@astellas.com

We want to assure you that Astellas and Astellas Gene Therapies remains committed to the safe and effective development of AT845 and to the patients and families affected by Pompe.

We will continue to provide updates on any significant developments in the AT845 investigational gene therapy development program as we are able. While we are committed to transparency, we also need to ensure that we preserve the data integrity of the clinical trial.

Sincerely,

Weston Miller, MD
Senior Medical Director,
Clinical Development

Hilary D'Anna
Senior Manager,
Patient Advocacy & Engagement