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Update to the U.S. Duchenne Community on the Ataluren (Translarna) NDA Review:

We have received a number of questions about the status of the ataluren new drug application (NDA) currently under review by FDA. We would like to take this opportunity to provide an update on the review status.

After many years and previous attempts, we were happy to share in October of last year that FDA accepted the NDA for ataluren for review. This was an important milestone as it was the first application for a therapy specifically for boys and young men with nonsense mutation Duchenne muscular dystrophy (nmDMD). Over the past 10 months we have been working with FDA to answer questions they had about the application approval package. Due to the historical circumstances of the ataluren application, the FDA does not have a specific date to reach a decision (also known as a PDUFA date). To date, a decision on approval of ataluren has not been reached, and the NDA remains under review.

The application includes the extensive evidence collected over more than a decade demonstrating that ataluren is safe and well tolerated and demonstrates meaningful evidence of disease slowing both in the short term, based on the results of the ataluren placebo-controlled trials and the long-term, based on results from the STRIDE registry. We believe the application provides strong evidence to support ataluren approval for all individuals affected by nmDMD.

We appreciate the letters many families have sent to the FDA to share personal stories that highlight the lack of treatment options specifically for those affected by nmDMD and thus the sense of urgency for ataluren approval. These personal stories uniquely enable FDA to hear directly from those most impacted by their approval decision. As FDA recently stated, “The patient community is an important voice, and the FDA will continue to listen and to respond to thoughts from the community impacted by DMD.”[1]

We will continue to work with the FDA and provide them with any additional information needed to help in their decision-making process. In addition, as we have more information about the approval application, we will keep the community informed. If you have any questions, please feel free to reach out to our patient engagement team at patientengagement@ptcbio.com.

Thank you for your continued partnership on this journey to deliver an approved therapy to those affected by nmDMD.

1 Feuerstein, July 28, 2025, STAT News, “FDA permits use of Sarepta Therapeutics’ Duchenne therapy in younger patients after short-lived halt”