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Update to the U.S. Duchenne Community:

We are pleased to share that the resubmission of the New Drug Application (NDA) for Translarna™ (ataluren) for the treatment of boys and young men with nonsense mutation Duchenne muscular dystrophy (nmDMD) has been accepted for review by FDA. This is a significant, positive step towards bringing this important treatment to those living with nmDMD in the United States.

The NDA resubmission is based on the findings of statistically significant benefit demonstrated on several key study endpoints in the overall intent-to-treat (ITT) population (N=359) of the global placebo-controlled trial, Study 041, as well as the findings of significant long-term Translarna treatment benefit in delaying time to loss of ambulation as captured in the STRIDE registry. We believe this evidence of short and long-term benefit, along with the established favorable safety profile of Translarna, supports approval.

The FDA has not provided a timeline for its review of the application due to the circumstances related to the last NDA review in 2016.

We are grateful for your steadfast support, advocacy and passion to advance care for those living with nmDMD and look forward to continued collaboration with the community and FDA. We will continue to keep you informed as we have more information regarding the progress of this application.