PTC Therapeutics, Inc.
500 Warren Corporate Center Drive
Warren, NJ 07059
www.ptcbio.com

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

Recent discussions with the U.S. Food and Drug Administration (FDA) have made clear that there are differences in data interpretation that cannot be successfully resolved to enable approval of ataluren (Translarna™) for the treatment of nonsense mutation Duchenne muscular dystrophy in the U.S. Despite the evidence of safety and effectiveness demonstrated across several clinical studies, FDA has shared that they view the data as insufficient to meet their threshold for approval. Accordingly, PTC has made the difficult decision to withdraw the resubmission of the New Drug Application for ataluren.

We understand that this outcome is devastating for the Duchenne muscular dystrophy community, the families who have participated in clinical trials for almost 20 years, and the families who continue to wait for a treatment option that addresses the underlying cause of nonsense mutation Duchenne muscular dystrophy.

Over the coming weeks, we will be determining next steps regarding supply of ataluren for those currently receiving therapy. We will provide more information once we complete our assessment. If you have any questions, please feel free to reach out to our patient engagement team at patientengagement@ptcbio.com.

We are humbled by the community's commitment and courage and deeply appreciate the many years of steadfast support in our shared mission. We are disappointed that, despite our best efforts, we have not been able to achieve FDA approval for ataluren.