PTC Therapeutics, Inc.

February 4, 2021

Dear Duchenne Community,

Your support and participation in the Translarna™ (ataluren) clinical development program has been incredibly important. We have been partners for over 20 years on this journey. Today, we are pleased to share the results on the dystrophin study (Study 045), which assessed dystrophin levels in patients with nonsense mutation Duchenne muscular dystrophy (nmDMD) treated with Translarna™ (ataluren) as recommended by the US FDA.

In this study, the effect of treatment with Translarna on full-length dystrophin expression was measured by two validated and sensitive assays: electrochemiluminescence (ECL), which was the primary endpoint, and immunohistochemistry (IHC), which was the secondary endpoint. Overall, there was an increase in dystrophin expression from baseline in both the primary and secondary endpoints, although it did not meet the p value of <0.05. However, when looking at the evaluable* population, there was a greater increase in dystrophin expression. We are pleased that over 80 percent of evaluable patients demonstrated an increase in dystrophin expression following Translarna treatment. Patients who received a longer duration of treatment due to COVID-19- related delays experienced a markedly greater increase in dystrophin expression, potentially suggesting that an extended duration of treatment may result in a greater biological effect, which is consistent with the long-term Translarna treatment benefit we have previously reported from our other clinical studies and our real-world STRIDE registry.

The data from the 045 study adds to the totality of Translarna data which includes demonstration of increased full-length dystrophin production, clinical benefit across multiple endpoints in several placebo-controlled trials, and real-world evidence of long-term clinical benefit on key aspects of disease progression.

We remain driven to deliver on our mission to provide access to this transformative therapy to the patients living with nonsense mutation Duchenne muscular dystrophy in the U.S. As a next step, we will be discussing these results and the collective clinical and real-world data with the U.S. Food and Drug Administration to determine if there is a potential accelerated path for approval in the U.S.

We will be hosting a conference call for the Duchenne community to discuss the Study 045 results and to answer questions. The call will take place on Thursday, February 11th at 12:30 pm ET. You can access the call by dialing (877) 303-9216 (domestic) or (973) 935-8152 (international) five minutes prior to the start of the webinar and providing the passcode 7714859. Please work with your patient advocacy group to submit questions.

We are grateful for your ongoing support and look forward to continuing our collaboration in the future.

Sincerely,

Stuart Peltz

Stuart Peltz, Ph.D.
Chief Executive Officer
PTC Therapeutics

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*20 patients were enrolled in the study, however data from two patients were not able to be analyzed due to noncompliance and sample quality.