Dear Friends,

On June 21st, we announced results from Study 041, our Phase 3 efficacy study of Translarna™ (ataluren) in boys and young men living with nonsense mutation Duchenne muscular dystrophy (nmDMD).

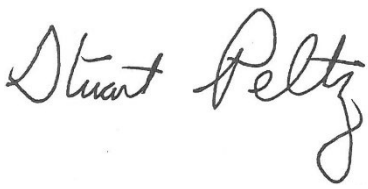
We are excited with the overall results of Study 041. It demonstrated that Translarna is the first disease-modifying treatment for Duchenne to show a statistically significant treatment benefit across the entire population and across multiple endpoints. In all endpoints measured,Translarna patients did better than placebo. In addition, the study results show slowing of decline for patients treated with Translarna, and fewer patients that lost ambulation. This is consistent with Translarna's long-term benefit that has been demonstrated in the STRIDE registry. While the study did not achieve a p value of less than 0.05 in the primary analysis of the subgroup >300 meters and >5 seconds stand to supine at baseline, the study did show benefit across the entire population of Duchenne boys. In addition, Translarna demonstrates a favorable safety profile with over 3000 patients treated to date.

The results from Study 041 adds to the totality of evidence demonstrating clinical benefit of Translarna in both clinical trials and in a real-world setting. We will be meeting with the regulators in the EU to discuss transitioning this to a standard approval and will meet with the FDA to discuss the next steps on how to bring Translarna to patients in the US.

We are incredibly grateful to all who have participated in ataluren clinical trials over the past 16 years. This milestone would not be possible without your participation and support.

For the most up-to-date information, please register on https://duchenneandyou.com so that we can continue to keep you informed.

Sincerely,



Stuart Peltz, PhD
CEO, PTC Therapeutics