



PTC Therapeutics, Inc.

100 Corporate Court
South Plainfield, NJ 07080
908.222.7000
www.ptcbio.com

October 25, 2017

Dear Duchenne Community,

As you might have seen, the Office of Drug Evaluation I of the FDA provided a Complete Response Letter (CRL) for the application to market ataluren in the United States. This means that the FDA believes ataluren cannot be approved at this time. We are extremely disappointed and we strongly disagree with the decision. We recognize that every delay means loss of function for those with Duchenne and there is an undeniable need to make ataluren available in the US. We are therefore planning on filing a formal dispute resolution request next week.

The dispute resolution process gives us an opportunity to appeal the letter issued by the Neurology division and have the decision on the application reviewed by the Office of New Products within the FDA.

We believe that ataluren benefits patients. We are inspired by your dedication and it drives us to continue our efforts to make ataluren available in the US. Your stories and experience inspire our work and will continue to be important for the decision makers to hear while we are in the dispute resolution process.

Many of you have inquired about continued access to ataluren through the extension trial and Siblings program. We have been supplying ataluren through these programs to over 150 boys in the US, some for as long as 10 years. We will continue with this access while in the formal dispute resolution process and will make a determination regarding the continuation of this program once the process has been completed.

We know that you have many questions that we will try to answer on a Community Call on Friday, October 27th at 12:00 PM EST. Please e-mail your questions in advance to patientinfo@ptcbio.com or share them with your patient advocacy organization. You can join the call by dialing (877) 303-9216 or (973) 935-8152; Conference ID: 5796459. Please dial in 5-10 min in advance.

Thank you for your partnership and dedication over the past 20 years and throughout this entire process. We need your continued support and tireless efforts to see this journey through.

Sincerely,

A handwritten signature in black ink that reads "Stuart Peltz".

Stuart Peltz, Ph.D.
President and CEO, PTC Therapeutics, Inc.