



PTC Therapeutics, Inc.

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

March 3, 2020

Dear Aniridia Community,

I would like to update you on the results of the STAR clinical study to treat Nonsense Mutation Aniridia. We have been working closely with investigators over the past four years on the development of ataluren. In January 2016 we initiated STAR clinical trial, which was conducted in two parts. The first part is a double masked and placebo controlled for 48 weeks. In the second part, all participants receive ataluren for an additional 96 -192 weeks. We also changed the trial from being a safety study to having a primary efficacy endpoint so that we can monitor both safety and efficacy. The reason we changed the trial was so that if the trial was able to show a difference, we would then be able to discuss this with the regulatory authorities. The primary endpoint measures the percent change from baseline to week 48 in Maximum Reading Speed (MRS) as measured by MNREAD acuity charts in patients 8 years of age and older. All patients have entered part 2 of the study and we have evaluated the data collected to date.

The results showed that the safety in this trial was similar to the known ataluren profile with overall safety profile similar between ataluren and placebo groups. Although not statistically significant, there were trends in the primary end points of Maximum Reading Speed (MRS) and the secondary endpoint of Reading Accessibility Index (ACC) show an improvement with ataluren treatment. The statistical significance was not achieved most likely due to the small sample size.

While, we are disappointed that the primary endpoint did not meet statistical significance, we will continue the evaluation of the participants currently enrolled in the study and will discuss the results with experts in the coming weeks after which a decision will be made on a path forward for the program in this indication. If you or a family member is participating in the trial, please continue to take ataluren as directed. If you have any questions, please direct those to the doctor that is conducting the trial.

We at PTC want to thank the community for your support and the active role you have played in both the trial and the natural history data collection. We are committed to working with the rare disease community to develop transforming therapies for neglected diseases such as Aniridia.

Sincerely,

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

Stuart Peltz, PhD
CEO
PTC Therapeutics, Inc.