

Update for patients and families on ataluren trial results
in nonsense mutation Duchenne/Becker muscular dystrophy

PTC Therapeutics and Genzyme Corporation are continuing to analyze results from the Phase 2b clinical trial of ataluren in patients with nonsense mutation Duchenne and Becker muscular dystrophy (nmDBMD). While the initial examination of the data showed that the primary endpoint of change in 6-minute walk distance did not reach statistical significance within the 48 week duration of the study, additional statistical analyses are necessary to fully understand the results. This will help determine the clinical and regulatory path forward for nmDBMD patients and for ataluren.

This landmark Phase 2b trial, which enrolled 174 boys and young men ages 5 to 20, showed that ataluren was well tolerated. No clinical trial patients discontinued treatment due to an adverse event. Thanks to the diligence of the investigators, patients and families, the trial was well conducted: Compliance with the drug regimen and study procedures was exceptionally high.

Now our work must continue. PTC and Genzyme are performing multiple additional analyses of data to better understand variations in trial results and, in conjunction with regulatory authorities, to identify the appropriate next steps for the ataluren program in nmDBMD. We understand that data from this study are important for ataluren clinical trial patients and the DBMD community as a whole. As we continue to collaborate with investigators, regulatory authorities, and patient advocates, we will provide updates on results.

We recommend that patients and families who participated in the Phase 2b and other ataluren clinical trials follow up with their investigator to discuss their individual results. We have provided treatment assignments to investigators that indicate whether a patient participating in the Phase 2b trial received placebo, ataluren low dose, or ataluren high dose. In addition, we have informed investigators that they may share trial-related information contained in local medical records with their patients.

We would like to extend our warm thanks and gratitude to everyone involved in this trial and in the Phase 2a trial and in particular the boys and young men who participated. Through their contributions, we have advanced our understanding of Duchenne/Becker Muscular Dystrophy for the benefit of the whole community. The trial has provided a wealth of data that will inform the design of future trials and the development of new treatment options.

If you have any questions regarding the ataluren clinical program you are encouraged to contact Genzyme Corporation at: eumedinfo@genzyme.com