

Genzyme Ataluren in nmDBMD Programme Update October 2010

Genzyme have developed this Programme Update as a way to communicate about ataluren and as part of our ongoing commitment in keeping the DBMD community informed. For more information please feel free to contact Genzyme's medical information department at eumedinfo@genzyme.com. *Please keep in mind that certain national regulations in Europe may prevent any form of communication between industry and patients (including provision of non-promotional information) so a physician may need to inquire on a patient's behalf.*

Following the recent presentation at the World Muscle Society congress of the final analysis of Phase 2b efficacy data, we understand that the DBMD community may have some questions on Genzyme's future plans for ataluren in its responsible geographies.

We would like to take this opportunity to reaffirm Genzyme's continued commitment to exploring the potential pathways forward for ataluren in nmDBMD in consultation with the relevant experts and regulatory authorities before the end of the year.

Given that Genzyme and PTC are working with regulatory authorities in different regions, the timing of interactions may differ depending on the regulatory framework for industry interaction and review of data.

Until we have more detailed discussions with regulatory authorities and they have had a chance to review the available data, it is difficult to predict a clear path forward. However once we have clarity, we will then be able to discuss next steps with you, including if and when expanded access programmes might be available and the possibility for additional studies of ataluren in DBMD.

Our main consideration is approval of ataluren so that all boys with nmDBMD may potentially benefit. We fully appreciate that this is a difficult situation and want to assure you that all of us at Genzyme remain committed to exploring the various options that may bring a therapy for these boys. As soon as there is more news to share we will update the DBMD community.