

**Genzyme ataluren in nmDBMD Programme Update:
ataluren clinical study
June 2011**

Genzyme has developed this Programme Update as a way to communicate about ataluren and as part of our ongoing commitment in keeping the Duchenne and Becker Muscular Dystrophy 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 the provision of non-promotional product information) so in some cases a physician may need to inquire on a patient's behalf.*

As announced in the May Programme Update, while Genzyme evaluates the available options for the ataluren in nonsense mutation Duchenne and Becker Muscular Dystrophy (nmDBMD) programme, we are working to initiate a follow-on clinical study that will provide equal access to all boys who previously participated in the ataluren nmDBMD clinical trials. We would like to provide you with additional details on this clinical study and the progress we have made to date towards initiation.

This study will be open to patients with nmDBMD who participated in the Phase 2b and Phase 2b Extension (007 and 007e) studies at a clinical trial site in Europe, Israel or Australia. It is designed to be an open-label study in which all patients receive ataluren (i.e. no placebo) and will be open to all previous study patients regardless of current clinical status. The study is planned to be conducted at the same sites that participated in the Phase 2b trials and is intended to further evaluate the safety of the "low-dose" ataluren regimen (10 mg/kg in the morning, 10 mg/kg in the afternoon, and 20 mg/kg in the evening). Study participants will be required to complete study visits every 12 weeks.

We want to make you aware that there are a number of operational, regulatory and logistical steps that must be completed for any clinical study before starting to enroll patients. Enrollment timelines will vary from country to country depending on national regulatory and ethics committee approval processes, however, on average it typically takes approximately 6 months for a study site to gain these approvals. We are in the process of selecting the contract research organization that will be responsible for managing the study on our behalf, and are currently working to finalize the agreement.

Genzyme currently does not have access to drug supply that is approved and available to support this study. So in parallel with the clinical activities noted above, Genzyme is working diligently to secure supply and obtain the necessary approval for use in the follow-on clinical study.

Based on these clinical and drug supply timelines and barring any new information, Genzyme anticipates the first patient to receive drug in the December 2011 timeframe. We will work internally, with the contract research organization and with the clinical sites to expedite these processes where possible.

Once we have further clarity on the next steps for ataluren in nmDBMD as well as any significant updates to the follow-on clinical study, we will update the community accordingly.