

Genzyme Ataluren in nmDBMD Programme Update March 2011

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 the provision of non-promotional product information) so in some cases a physician may need to inquire on a patient's behalf.*

In our efforts to determine next steps in moving the nmDBMD programme forward, Genzyme has conducted a thorough evaluation of all the available data accumulated to date on ataluren.

Because of the complexity of the data set, we recently obtained formal scientific advice from European regulatory authorities. The discussions at these meetings were productive and their recommendations on how to further strengthen the data package were extremely valuable.

As a result of our own evaluation and the feedback we received from European regulatory authorities, Genzyme has initiated additional data analyses, which aim to further understand the unexpected post hoc results of the Phase 2b study that suggested efficacy at the lower dose but not at the higher dose. We expect that this extra work will inform how best to proceed with our regulatory filing strategy and timing.

We will continue to provide updates on a regular basis to the nmDBMD community on our progress towards submission for regulatory review.

Our goal is to gain approval of ataluren and provide access to treatment so that all boys with nmDBMD may potentially benefit. We appreciate your continued patience and understanding during this period of time and are highly appreciative of the community's support as we build a more complete understanding of ataluren as a treatment for nmDBMD.