

Genzyme Ataluren in nmDBMD Programme Update December 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 eumedinfor@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.*

Genzyme and PTC continue to work diligently with regulatory authorities in different regions regarding ataluren. Due to regional variances, the timing of these interactions, procedures and pathways differ depending on the regulatory framework for interaction between industry and authorities.

As you are aware, these differences have been highlighted by recent developments in the USA, whereby PTC, with the support of the US FDA, implemented an open label safety study as an alternative to supporting individual patient IND requests (a US specific mechanism to gain access to non-approved treatments). Only nmDBMD patients who previously participated in ataluren trials at U.S. sites are eligible for this U.S. program. This particular safety study is not available in Europe.

We recognize that European families may be frustrated by a perceived lack of progress in Europe in comparison to the US, and the difficulties in setting expectations around the potential and timing of nmDBMD patients having access to ataluren. Genzyme wants to assure you that we have been active in evaluating how best to advance the programme and that we are making progress. We have recently initiated meetings with EU regulatory authorities to seek scientific advice on the data and potential paths forward, including the possibility of pre-approval access programmes. Upon receipt of their formal feedback we will be in a position to complete our evaluation and communicate next steps.

We appreciate your continued patience and understanding during this period of time as we diligently work to complete our evaluation to determine the path forward. Our commitment remains to update the nmDBMD community in the first quarter of 2011 and our goal is to make decisions that are in the best interest for this community.