

## Genzyme ataluren in nmDBMD Programme Update 2nd May 2011

Genzyme has 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](mailto: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.*

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Over the past months, Genzyme has been working on increasing our understanding of ataluren as a potential treatment for boys with nonsense mutation Duchenne Becker Muscular Dystrophy (nmDBMD). Due to complexity of the data, we sought formal scientific advice from European regulatory authorities in order to decide on the best path forward for the ataluren programme for nmDBMD in Europe. As a result of the feedback we received, Genzyme has been actively conducting additional analyses on available data of ataluren. Once we have completed these analyses we will be in a better position to decide our regulatory filing strategy and timing for nmDBMD in Europe.

As part of our continuing assessment, Genzyme plans to initiate a follow-on clinical study of ataluren in nmDBMD patients who previously participated in the clinical trials in Europe, Israel and Australia. All patients who have previously participated in the clinical trials with ataluren in nmDBMD, irrespective of their current clinical status, will be eligible to participate.

We believe that this clinical study will allow for the collection of additional information on ataluren in nmDBMD while providing access to ataluren to all patients who have been involved in earlier clinical trials.

Currently we are working to develop the timelines and design for this clinical study and we plan to update the community, with these additional details in the very near future. We are reaching out to the original trial investigators to make them aware of this new development and they may be in touch with eligible families directly as well.

Genzyme takes our commitment of providing transparent, regular updates to the community very seriously. Consequently we have chosen to share this important new development as quickly as possible. We understand that we may not have all of the details of this clinical study available to share with you at this time and that you may likely have questions that we may not yet be able to address. However, we believe that sharing this news is important and will work to provide more information as it becomes available.

Our goal remains to obtain approval of ataluren such that all boys with nmDBMD may potentially benefit. We very much appreciate your continued understanding during this period of time, as we clarify the details of the clinical study and we will keep you properly informed as this occurs.