**Genzyme ataluren in nmDBMD Programme Update**
**September 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.*

---

As reported in our June Programme Update, Genzyme has been actively exploring various options in our efforts to determine appropriate next steps for the nmDBMD programme. While we have been very pleased with our long-standing relationship with PTC, as a result of our recent portfolio alignment process, we would like to inform the community that Genzyme has decided to restructure our relationship with PTC for the ataluren nmDBMD programme. We will, however, retain an option to reengage the collaboration for non-nmDBMD indications, which reflects our belief in the potential of this approach for the treatment of nonsense mutation genetic disorders.

Also in our June Programme Update, Genzyme informed the nmDBMD community that we initiated start up activities for a follow-on clinical study for patients who participated in earlier ataluren nmDBMD clinical trials. Moving forward, PTC now has full, global responsibility for the clinical, regulatory and commercial strategy of ataluren in nmDBMD. Genzyme will work closely with PTC to ensure an efficient transition of all DBMD related activities to PTC. Questions relating to the follow-on safety study follow-on clinical study for patients who participated in earlier ataluren nmDBMD clinical trials should be directed to PTC.

If you have any questions relating to this announcement or the follow-on safety study, we would kindly ask that you forward these directly to PTC who are best placed to address them. PTC can be contacted either by e-mail at PatientInfo@ptcbio.com or by telephone +1 866 282-5873.

Genzyme is deeply impressed by the unwavering dedication, support and collaboration within the community on the behalf of families affected by DBMD, particularly the boys living with this disease. We have greatly appreciated the community's patience and engagement in ongoing dialogue during our involvement with the ataluren nmDBMD programme. Even though Genzyme will no longer be involved with ataluren in nmDBMD, we would like to assure you that we remain committed to the neuromuscular patient community and will continue to look for innovative, transformative therapies for those with unmet medical needs.