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January 17, 2009

Dear Pompe Patient,

The AMDA is writing to our Pompe patient community to let everyone know that the AMDA has been working closely with the IPA and Genzyme in order to get communication out to our patient population about the status of the Myozyme supply issue.

In July 2008, Genzyme Corporation issued a notification that the worldwide Myozyme supply would be tight in 2009 until European Commission approval of a larger scale, 4000L production facility in Belgium. Genzyme also stated that receiving approval for Myozyme produced at the 4000L manufacturing scale in the first half of 2009 would greatly improve its ability to return to normal levels of inventory.

The IPA worked with Genzyme to form an international Myozyme Stakeholders Working Group (MSWG) in August, 2008. This group was comprised of members of the IPA, Genzyme, and several physician experts in the management of Pompe disease from the US and Europe. The goal of the MSWG was to develop practical guidance for clinicians and patients. The complete guidance documents created by the MSWG are included in this mailing.

Most significantly, the guidance documents recommend that all adult Pompe patients miss one infusion at the end of January 2009 and another infusion each month thereafter until the period of tight supply ends. It is hoped that this action will be sufficient to preserve drug inventory until Myozyme from the new production facility in Belgium is approved in Europe, and that no further infusions will need to be missed. Our hope is that only a small number of missed infusions will be required.

Implementation of this modification to treatment schedules beginning in January for adults is essential; this will help ensure that no infant or child will miss an infusion due to potential shipping interruption or delay during this finite period.

Your health care professionals are advised to take this guidance into account when exercising their clinical judgment. If there are genuine concerns about modifying the treatment schedule of any severely affected patients, physicians should contact Genzyme Medical Information.

Please don't hesitate to contact the AMDA if you have any concerns not addressed in the information provided to you.

Best regards,

Tiffany House  
President  
AMDA