



2nd March 2009

Dear IPA Affiliate,

IPA Statement: The End of Myozyme Supply Restrictions

You will no doubt have heard the wonderful news that Myozyme from the Belgian production facility has been approved for patients within the European Community (see the IPA website for the press release). Another great success has been the adoption of the Myozyme Stakeholder's Working Group (MSWG) guidelines by the majority of the Pompe community. A third strand to this story was the initiation of a trial of the Belgian product involving patients in the Netherlands; designed to reduce the global demand on the Myozyme produced in the United States.

The result of these actions is that Myozyme inventory is sufficient to end the restrictions that were put in place in February. Thankfully most patients will only have missed one infusion and should not be required to miss any more. Genzyme have already written to all treating physicians and patients should be hearing from their treatment centres very soon.

Genzyme had been constructing and testing the Belgian production facility for the last five years. I also know that the company has put in a tremendous effort over the last year to win early approval by EMEA and to ensure that the patient community was protected as much as possible from the effects of a supply shortage. A shortage brought about by the unpredictably high number of patients on therapy so soon after its approval and the very high dose needed per patient, when compared to other lysosomal storage disorders. We should commend Genzyme for their valiant efforts.

I would also like to thank the Patient community for their acceptance of the guidance that the MSWG sent out in February, we were never certain whether the guidelines would be fully adopted, but we have been very pleasantly surprised. By adults missing just one infusion Genzyme was able to guarantee uninterrupted supplies to all children and infants as well as several severely affected adults throughout the world.

I would also like to give my personal thanks to all the members of the MSWG who appreciated the need to develop an effective communication plan that would inform patients, physicians, health providers and the media in order to manage a tight supply situation should it arise. They have all dedicated many hours of their time to develop effective guidelines, often adapting to circumstances that changed by the week.

Of course, without the great understanding of the staff at EMEA and their appreciation of the urgency required, we would be in a very different situation. So our thanks also go out to the Committee for Medicinal Products for Human Use (CHMP) who worked so hard to give an accelerated approval to the 4000 litre product.

To ensure that lessons learned from this episode do not disappear into Pompe folklore, the IPA will be working with Genzyme and health professionals to gather information and document it for future reference. In the near future we plan to write to the global Pompe community to ask how the drug shortage affected them; what went well and what could have been better. In the meantime, if you would like to send your comments to the IPA, please do so by contacting either Marsha or Paula; their contact details are given overleaf.

Thank you all,

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National Patient groups that support the Pompe community can be found at:

<http://worldpompe.org/index.php/affiliates>