International Pompe Association
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The Netherlands

www.worldpompe.org

14th January 2008

Dear IPA Affiliate,

IPA Statement Regarding the Management of Myozyme Supply

Since the first market approval of Myozyme, the IPA has liaised directly with Genzyme LSD Therapeutics to insist that an adequate inventory of the product was kept to ensure continuous supply to all patients receiving the treatment; whether through their commercial or compassionate-use programs. Regretfully, in July 2008 Genzyme released a notification that supply would be tight in 2009 until a larger-scale production facility in Belgium is approved by the European Medicines Agency (EMEA).

Ever since that time, the IPA has urged Genzyme to find ways of increasing Myozyme production and, with the prospect of a tight supply, has worked very closely with Genzyme and expert physicians to prepare advisory guidelines for treating physicians. Playing an important role in the Myozyme Stakeholder's Working Group (MSWG), the IPA has helped to formulate guidance for physicians, carefully designed to protect the most vulnerable patients. At this time, full protection can only be achieved by a reduction in demand for Myozyme from those patients who can tolerate a temporary interruption in their treatment.

I have attached a copy of the resulting guidelines which recommend that adult patients should miss one infusion at the end of January 2009 and should miss 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.

Genzyme is distributing these guidelines to treating physicians in all countries outside the European Union (EU) - within the EU a separate letter is being sent with a similar message from the European Medicines Agency (EMEA); however there may be circumstances where the message does not reach all patients. The IPA would ask to you to distribute this information through your local networks to reach patients who will be affected by the guidelines, also treating physicians and any other appropriate healthcare workers (e.g. homecare nurses) that are involved with Myozyme infusions in your locality. The aim is to encourage global cooperation in order to protect the most vulnerable patients in the Pompe community, especially children and infants.

If, after reading the guidelines, you have further questions, please contact either your local Genzyme office or the IPA contacts in Europe or in the USA. The IPA contact information is listed on our website and on the reverse of this letter. Our website also contains Frequently Asked Questions that you may find useful.

I can give my personal assurance that Genzyme staff have worked tirelessly during the last six months to try to minimise the impact of tight supply of Myozyme. The other members of the MSWG have given many hours of their time to develop an optimal strategy for coping with this period of constraint. It is now up to the Pompe community of physicians and patients to give their full cooperation to ensure a successful outcome. After this short and difficult period we hope that inventories will return to levels that will assure uninterrupted treatment for the whole Pompe population.

Thank you for your assistance

Allan Muir, Chairman, International Pompe Association

IPA Contact Information

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National Patient groups that support the Pompe community can be found at:
http://worldpompe.org/index.php/affiliates