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BREAKING NEWS ON 160 LITER MYOZYME SUPPLY

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Dear United States Pompe Community,

As most of you are aware, in the United States there are two commercial products manufactured by Genzyme to treat Pompe Disease: Lumizyme (which is produced at the 4000 liter scale and sold as Myozyme everywhere else in the world) and Myozyme (which is produced at the 160 liter scale and only used in the United States).

As has been mentioned previously, Genzyme's ability to produce the 160 liter Myozyme is limited due to the size of the bioreactors. Unfortunately, we have now reached a point where Genzyme can no longer guaranty uninterrupted access to the 160 liter Myozyme because of these production constraints.

On the AMDA (www.amda-pompe.org) and IPA (www.worldpompe.org) websites a Pompe Program Update and Fact Sheet that describe the supply situation and the steps that Genzyme is taking to provide an avenue to access treatment for those who are currently on the 160 liter Myozyme. More specifically, Genzyme will be initiating a clinical trial called ADVANCE. In this trial, patients over the age of 12 months will be transitioned to the 4000 liter product, which will allow Genzyme to preserve enough product to ensure that those under 12 months of age will have uninterrupted access to the 160 liter Myozyme.

While I know there will be many questions over the next few weeks and months, I wanted to take this opportunity to reassure the Pompe Community that the AMDA is aware of the situation and I have been in close contact with Genzyme and with Dr. Priya Kishnani of Duke University regarding this situation. To that end, Dr. Kishnani has asked me to pass along the following message:

> *I am writing today in an effort to provide some clarity around the topic of treatment for Pompe disease in the US. As most of you are aware, there are currently two drugs available for the treatment of Pompe disease. Lumizyme – which is alglucosidase alfa produced at the 4000 liter scale and Myozyme which is alglucosidase alfa produced at a 160 liter scale. These variations in production scale are related to the volume of drug that Genzyme must manufacture in order to meet the needs of the Pompe community in the US. In the US, Lumizyme is used to treat patients who are 8 years of age and older and Myozyme is used to treat patients under the age of 8 with some exceptions for patients over 8 who were born with infantille onset Pompe disease or who have cardiac hypertrophy in the late onset spectrum.*
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> *Outside of the US, this is not the case. Lumizyme (alglucosidase alfa produced at the 4000L scale) is approved and referred to as Myozyme and is being used to treat **all** patients with Pompe disease, regardless of age. Lumizyme is currently being used to treat patients of all ages in over 50 countries. As many of you know, I have had the privilege of working with infants all over the world who are affected by Pompe disease and in my experience these babies are doing well on the 4000L product; additionally, data from the Taiwan newborn screening studies has been reassuring with regard to infants on Lumizyme.*
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> *As with any biologic material there could be some patients that may have some side effects, but based on global experience, I feel they would be quite manageable medically. One of the requirements of any clinical study is close monitoring and supervision and so of course all patients will be monitored very carefully as they make this transition from enzyme that is made at the 160L scale to enzyme made at the 4000L scale. Our team at Duke is available to answer and questions or concerns should they arise.*

While this situation is certainly not ideal, Genzyme is working hard to try to ensure that patients will not experience missed infusions and I will continue to work closely with them and Dr. Kishnani to bring you the most up-to-date information possible.

Also, Genzyme will be hosting a US Pompe Community Town Hall (as in mentioned in the Program Update) to address this situation and provide more information on Tuesday, January 31, 2012 at 7 pm (EST). If you are interested in participating in this Town Hall, please contact me (TiffanyLHouse@aol.com) or Marsha Zimmerman (marzim50@gmail.com) and we will provide you with the call-in information.

If, after reading through the attached material, you have any questions or concerns, please contact me or Marsha and we will do our best to get you the answers you need.

Tiffany House
AMDA President