

## Genzyme Pompe Community Program Update

April 15th 2015

Genzyme is pleased to recognize the Second Annual International Pompe Awareness Day. As a longtime partner of the International Pompe Association (IPA), we have a shared mission of raising Pompe disease awareness globally and commend the IPA for the creation of this important event. In celebration of International Pompe Awareness Day, we wanted to share two new initiatives with the community.

First, Genzyme is very pleased to announce the publication of a 10-year Pompe Registry Report for patients. This report is available to those patients who are enrolled in the Pompe Registry with a signed consent form, and can be obtained through treating physicians. The report will be made available in the coming weeks and months in different countries around the world subject to local approvals.

The Pompe Registry was created so that the medical community could have a greater understanding of Pompe disease and recognize clinical signs of patients with the disorder. This Registry is the world's largest collection of data on Pompe patients and this critical information has given us tremendous new insights into Pompe disease, specifically in the areas of diagnosis and disease management.

The goals of Genzyme's first ever Pompe Patient Registry Report are to provide a "then-and-now" historical comparison of the Pompe Registry and a look at how the Registry has changed; show how the Registry has grown over time and how it has contributed substantially to our current and ongoing understanding of Pompe disease; and exemplify why it is so very important for patients of all ages with Pompe disease around the world to be enrolled and actively participate in the Registry with their healthcare teams. Key findings from this report include:

- Since it was started in 2004, enrollment in the Pompe Registry has grown steadily each year. As of May 2, 2014 (the date through which data are reported here unless otherwise noted), a total of 1,292 patients worldwide were enrolled in the Pompe Registry.
- In 2014, the overall mean age at first symptom in patients with late-onset Pompe disease was 27 years. The mean age at diagnosis for these patients was 34 years. Therefore, there was a clear diagnostic delay in late-onset patients.

We would like to thank patients and their families for taking part in the Pompe Registry. Without your willingness to participate and share your clinical data, our understanding of

Pompe disease would be much more limited. We also would like to acknowledge the Registry Board of Advisors for their guidance and direction, and the clinicians and their staff at sites around the world, who collect and enter data into the Registry. For additional information about the Pompe Registry, contact your physician or visit [www.registrynxt.com/Pompe](http://www.registrynxt.com/Pompe).

Also, over the past several months, Genzyme has been working to develop a television segment dedicated to increasing Pompe Awareness, sharing perspectives of patients and care givers. This program aired nationally in the United States on the Lifetime Television Cable Network on April 15<sup>th</sup>, and will be shown to Genzyme employees throughout the day on April 15<sup>th</sup>. The program features Dr. Barry Byrne, who provided background on Pompe disease and was joined later in the program by Tiffany House, head of the Acid Maltase Deficiency Association and current head of the International Pompe Association, and David Hamlin, head of the United Pompe Foundation. Tiffany and David shared their personal Pompe experiences both as a patient and a parent, respectively, and also elaborated on the history and mission of their respective patient organizations. When developing this program we all felt that it was critical to go beyond the traditional scope of increasing awareness about the disease by featuring patient association leaders, elevating the important work of patient associations like the AMDA and the UPF.

Genzyme is a proud member of the Pompe community and today, on International Pompe Awareness Day, our organization celebrates all the progress made in Pompe disease and remains committed to the work left to be done.