

Key milestones in Genzyme's effort to develop Myozyme, the first and only approved treatment for Pompe disease



**1998
to
2002**

In the early years, Genzyme focused on efforts to advance promising research involving four different potential treatments for Pompe disease:

- a candidate developed internally at Genzyme in collaboration with researchers at Duke University in the U.S. and Erasmus Medical Center in The Netherlands.
- a candidate developed in conjunction with Pharming Group NV, a company in the Netherlands.
- a candidate originally developed by Synpac, a company based in North Carolina.
- a candidate originally developed by Novazyme, a company in the U.S. that was acquired by Genzyme in 2001.



2002

Genzyme completes a major analysis of the research findings for all four candidates in an effort nicknamed "The Mother of All Experiments." This analysis indicates that the product candidate developed internally at Genzyme shows the greatest promise for efficacy in the treatment of Pompe as well as for successful production. Research efforts related to the other product candidates are eventually terminated and Genzyme focuses on the successful development of this product, known as Myozyme.



2003

Genzyme initiates clinical trials for patients with Pompe disease. The research involves a global effort to identify infants with Pompe disease – many of whom are severely sick.



2004

In May the clinical trial for Myozyme is fully enrolled, thanks to the efforts of hundreds of Genzyme employees, doctors and other health professionals, patient groups and the heroic families affected by Pompe disease around the world.

2005

Genzyme reports positive phase 3 clinical results for Myozyme. The company files for regulatory approval for Myozyme in Europe and the U.S.

2006

Myozyme is approved for the treatment of Pompe disease in the U.S. and in Europe. Genzyme seeks approval to produce the drug in larger batches to meet demand.

2007

In the U.S., Myozyme is reserved for treatment patients 17 years and younger. The drug produced in larger batches is submitted for regulatory approval and is provided free-of-charge to adults with Pompe disease.



Present

More than 1,000 Pompe patients around the world receive treatment with Myozyme.