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## **FDA Withholds Approval of Zavesca as Treatment for Niemann-Pick Disease Type C**

*Though a setback, announcement is not the end of the road*

**Fort Atkinson, WI (March 15, 2010)** – The National Niemann-Pick Disease Foundation, based in Fort Atkinson, has received word that the U.S. Food and Drug Administration (FDA) did not grant outright approval of the drug Zavesca (miglustat) as a treatment for the rare, genetic and terminal condition, Niemann-Pick Disease Type C (NPC).

Rather, the FDA issued a “Complete Response Letter,” allowing Actelion Pharmaceuticals, maker of Zavesca, to continue to collect and submit data which might more clearly show the positive impact the drug has on individuals with NPC. A difficulty in developing a drug for treatment of a rare disease such as NPC is the small number of patients involved, and the accompanying challenges in collecting compelling amounts of data.

The FDA’s Endocrinologic and Metabolic Drug Advisory Committee met in January to hear statements and testimony from scientists, clinicians, and families affected by Niemann-Pick Disease Type C. The panel recommended approval of Zavesca, and since then, the FDA had been reviewing data to make its determination regarding the use of Zavesca in NPC patients.

Zavesca is approved for the treatment of progressive neurological manifestations in adult and pediatric patients with NPC in the European Union, South Korea, Brazil, Russia, Australia, and most recently, Canada. (Canada approved Zavesca on March 4, 2010.)

Insurance coverage of Zavesca for NPC patients in the U.S. could have been expected to improve if the FDA had issued approval. The drug costs approximately \$159,000 a year per patient.

Actelion has indicated the company remains committed to moving forward in working for FDA approval of Zavesca for patients with NPC. Zavesca is not a cure for NPC, but it has shown promise in treating symptoms related to this neurodegenerative disease and in slowing its progression for some patients. Had the FDA given its approval, Zavesca would have been the first authorized treatment for NPC in the U.S.

Zavesca has been prescribed in the U.S. since 2003 for treatment of patients with Type 1 Gaucher Disease. As a result of the FDA’s Tuesday announcement, U.S. physicians must continue to prescribe Zavesca “off-label” for NPC.

One of more than 50 lysosomal storage diseases, NPC causes accumulation of fats in all cells, but most significantly, in the liver, spleen and brain. Accumulation of these fats disrupts cell function, leading to severe physical and neurological deterioration, including the loss of the ability to walk, speak and swallow. NPC is always ultimately fatal.

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## **About the National Niemann-Pick Disease Foundation (NNPDF)**

The National Niemann-Pick Disease Foundation (NNPDF) was established in 1992 to fund family support services and research into Niemann-Pick Disease. Since then, the Foundation's membership has grown to over 350 families, and over \$4.3 million has been applied toward research. As a result of this research, the genes responsible for Niemann-Pick Disease have been identified, and research continues, seeking treatments and a cure for Niemann-Pick Disease.

In addition to its work within the United States, the NNPDF provides support to the Canadian Chapter of the NNPDF. The NNPDF also provides education, referrals and advocacy to a broad international membership via its Web site at [www.nnpdf.org](http://www.nnpdf.org).

For more information about the National Niemann-Pick Disease Foundation, visit [www.nnpdf.org](http://www.nnpdf.org) or contact the NNPDF by telephone at 920-563-0930 or by email at [nnpdf@nnpdf.org](mailto:nnpdf@nnpdf.org).

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