



Canadian Chapter
National Niemann-Pick Disease Foundation
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Canada Approves Zavesca as Treatment for Niemann-Pick Disease Type C First Authorized Treatment in Canada for Fatal Disorder

Durham, Ontario (March 24, 2010) – Health Canada recently announced its approval of the drug Zavesca (miglustat) as the first authorized treatment for neurological symptoms of the rare, genetic and terminal condition, Niemann-Pick Disease Type C.

Zavesca is not a cure for Niemann-Pick Disease Type C (NPC), but it has shown promise in treating symptoms related to NPC and in slowing the progression of the disease for some patients. The product's monograph will read, "Zavesca is indicated to slow the progression of some of the neurological manifestations in patients with Niemann-Pick Type C disease."

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 physical and neurological deterioration, including the loss of the ability to walk, speak and swallow.

Produced by Actelion Pharmaceuticals, Zavesca is also used for treatment of patients with Type 1 Gaucher Disease, the most common lysosomal storage disease.

As the first-ever approved treatment for Niemann-Pick Disease Type C in Canada, Zavesca provides a ray of hope for families who are desperate to extend and improve their children's quality of life.

About the Canadian Chapter of the National Niemann-Pick Disease Foundation (CCNNPDF)

The Canadian Chapter of the National Niemann-Pick Disease Foundation (CCNNPDF) provides family support services and promotes research into Niemann-Pick Disease. In collaboration with the National Niemann-Pick Disease Foundation (U.S.), the foundations' combined 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.

The CCNNPDF will host the 18th Annual National Niemann-Pick Disease Family Support and Medical Conference at the Delta Chelsea Hotel in Toronto, August 5 – 8, 2010. Families from Canada, the United States, and several other countries will attend the conference to hear the latest information from Niemann-Pick Disease researchers and to network with and support other families struggling with the disease.

For more information about the Canadian Chapter of the National Niemann-Pick Disease Foundation, visit www.nnpdf.ca , or call 920-563-0930, or email nnpdf@nnpdf.org .

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