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Local Mom Testifies to FDA Advisory Committee Zavesca Under Consideration as Treatment for Niemann-Pick Disease Type C

Fort Atkinson, WI (January 14, 2010) – Barb Vorpahl of Fort Atkinson testified in front of a U.S. Food and Drug Administration (FDA) advisory panel during its recent review of the drug miglustat (Zavesca) for treatment of Niemann-Pick Disease Type C. Vorpahl and her husband, Gary, are the parents of Stacey Vorpahl, who passed away in 2004 at the age of 19 from the effects of NPC. Mrs. Vorpahl is Board Chair of the National Niemann-Pick Disease Foundation (NNPDF) based in Fort Atkinson, WI.

The FDA's Endocrinologic and Metabolic Drug Advisory Committee met in Silver Spring, Maryland, to consider the new drug application of Zavesca for use in patients with Niemann-Pick Disease Type C (NPC). The panel of medical and clinical experts reviewed data and heard statements and testimony from scientists, doctors and NPC families in a daylong review January 12. Zavesca, produced by Actelion Pharmaceuticals, is already being prescribed in the U.S. for patients with Type 1 Gauchers' Disease, another rare disorder.

To date, there is no authorized treatment in the United States for NPC, a lysosomal storage disease causing accumulation of fats in the cells of the liver, spleen and brain. When cell function is blocked, physical and neurological deterioration occurs, including the loss of the ability to walk, speak and swallow. NPC is a rare, genetic disease, and is always terminal.

Doctors in the U.S. can currently prescribe Zavesca "off-label" for NPC, but Actelion needs FDA clearance to market the drug specifically for that use. Further, insurers are reluctant to pay for the drug for NPC patients without the FDA approval. The drug costs approximately \$159,000 a year per patient.

Zavesca is not a cure for NPC, but it has shown promise in treating neurological symptoms related to the disease and in slowing the progression of the disease for some patients. As Vorpahl testified to the committee, Zavesca provides a ray of hope for families who are desperate to save the lives of their children.

Many NNPDF member families, including the Vorpahls, submitted letters to the advisory panel in advance of the hearing, regarding the impact Zavesca has had on their family member, or what the availability of Zavesca might have meant for a loved one already lost to NPC.

Vorpahl addressed the panel on behalf of NNPDF families, along with Cindy Parseghian of the Ara Parseghian Medical Research Foundation (APMRF) and Phil Marella of Dana's Angels Research Trust (DART).

Dr. Isaac Kobrin, Actelion's chief medical officer, told the panel, "This is a relentlessly progressive disease. We clearly see a change in the progression rate (with Zavesca). It's a very clear signal."

Weighing the risk of the drug versus potential benefits, Katherine Flegal, a senior research scientist at the Centers for Disease Control and Prevention, stated, "The risk from the drug is not devastating, but the disease is devastating. I think patients deserve an opportunity to see if they can benefit."

After some consideration, the advisory committee voted 10-3 to recommend to the FDA that Zavesca be approved for treatment of the symptoms of NPC. The FDA will consider the committee's recommendation, and is expected to make a decision by March 10.

If the FDA approves the use of Zavesca for NPC, it will be a historic step, as this would be the first authorized treatment for the symptoms of NPC in the United States.

About the National Niemann-Pick Disease Foundation (NNPDF)

The NNPDF was established in 1992 to raise money for family support services and research. 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 gene responsible for Niemann-Pick Disease has 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 audience via its Web site at www.nnpdf.org.

For more information about the National Niemann-Pick Disease Foundation, visit www.nnpdf.org or contact the NNPDF by telephone at 920-563-0930 or by email at nnpdf@nnpdf.org.

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