



3/16/2010 11:25:00 AM

FDA: No drug for NPC yet

Despite patient advocates' high hopes, the U.S. Food and Drug Administration has not granted outright approval of using the drug Zavesca to treat a rare, genetic disease that claimed the life of a Fort Atkinson teen.

The National Niemann-Pick Disease Foundation, based in Fort Atkinson, has received word of the FDA's decision regarding Zavesca (miglustat) as a treatment for Niemann-Pick Disease Type C (NPC).

The FDA issued a "complete response letter" that does, however, allow Actelion Pharmaceuticals, the maker of Zavesca, to continue to collect and submit data that might more clearly show the positive impact the drug has on individuals with NPC.

The FDA's Endocrinologic and Metabolic Drug Advisory Committee met Jan. 12 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.

Among those who testified in January was Barb Vorpahl of Fort Atkinson, whose daughter, Stacey, died of the illness in 2004 at age 19. She noted that the Niemann-Pick Disease Foundation first heard of Zavesca a decade ago. In the 10 years since, 146 children and adults that the foundation knows of have died from Niemann-Pick Type C.

"This may not sound like many when compared to other well-known diseases, but in our small Niemann-Pick community, with only about 343 known cases, that number is staggering," Vorpahl, NPC Foundation president, told the FDA advisory panel.

Since then, a number of Niemann-Pick patients have had access to Zavesca through clinical trials and have reported very hopeful results. Vorpahl said that although these patients did not experience a cure, Zavesca did appear to slow the "relentless progression" of the disease, giving them months, even years, to enjoy a higher quality of life.

"You can't imagine our excitement when we started hearing from parents that they were actually seeing improvements in the symptoms of this disease," Vorpahl told the committee Jan. 12. "We were overjoyed to hear that in siblings and others being treated with Zavesca early enough it was actually holding off the symptoms ... This drug is making a real difference."

Vorpahl and her husband, Gary, joined with other Niemann-Pick families to form the foundation after Stacey was diagnosed at the age of 2. The foundation has now been around for 18 years, and Vorpahl has continued as a board member throughout, serving as chairperson for seven of those years.

One of more than 50 lysosomal storage diseases, Niemann-Pick Disease-Type C causes accumulation of fats (cholesterol) 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 always is ultimately fatal.

A difficulty in developing a drug for treating a rare disease such as NPC is the small number of patients involved, and the accompanying challenges in collecting compelling amounts of data.

Zavesca has been prescribed in the U.S. since 2003 for treatment of patients with Type 1 Gaucher's disease, the most common of the lysosomal storage disorders. As a result of the FDA's Tuesday announcement, U.S. physicians must continue to prescribe Zavesca "off-label" for NPC.

Zavesca is approved for treating progressive neurological manifestations in adult and pediatric patients with NPC in the European Union, South Korea, Brazil, Russia, Australia and - as of March 4, 2010 - Canada.

The drug costs approximately \$159,000 a year per patient. Insurance coverage of Zavesca for NPC patients in the U.S. was expected to improve had the FDA accepted the advisory panel's recommendation and issued approval.

Actelion has indicated that 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.

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 website 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 sending an e-mail to nnpdf@nnpdf.org.