

A Niemann-Pick Milestone

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Fort Atkinson, Wisconsin*

To say we are excited that the FDA could be close to approving a treatment for Niemann-Pick Disease-Type C is an understatement. We are ecstatic.

Fort Atkinson's Barb Vorpahl recently testified before a Food and Drug Administration advisory committee, urging approval of the drug Zavesca to help victims of the rare disorder that claimed the life of her daughter, Stacey, six years ago. It is hoped the FDA will make its decision yet this month.

Niemann-Pick Disease Type C causes progressive deterioration of the nervous system by blocking the movement of cholesterol within the body's cells. Building up in vital organs, it eventually threatens the most basic bodily functions, such as breathing and swallowing.

Most readers undoubtedly know how Barb and Gary Vorpahl joined with other parents to form the National Niemann-Pick Disease Foundation after Stacey was diagnosed at age 2. Eighteen years later, the Fort Atkinson-based foundation is stronger than ever, and Barb continues to be a strong leader on its board.

The foundation first heard of Zavesca as a treatment for Gaucher's disease, the most common of 40 lysosomal storage disorders that include Niemann-Pick. In the decade since, at least 146 children and adults with the disorder have died, an atrociously high number considering there are only 343 known cases of Niemann-Pick Disease-Type C.

FDA approval of Zavesca for this use, then, would be a godsend. Clinical trials have shown it to slow the relentless progression of the disease; in fact, some children on the drug have experienced no physical decline in three to four years. And there have been some small gains in swallowing and walking recorded, as well.

The drug costs approximately \$159,000 per year and FDA approval could lead to insurance coverage that would enable the average family to be able to afford this drug ... and hopefully allow their loved one to live more years with a much higher quality of life.

We've no doubt this day would not have come had it not been for families like the Vorpahls, the foundation they helped create and the countless friends and strangers who supported them through fundraising and prayers. From Mustang rides and golfing tournaments to silent auctions, church suppers and outright donations, the greater Fort Atkinson community has played a huge role in backing the foundation's efforts to provide education and support to NPDC families and money to finance research toward finding treatment and even, some day, a cure.

There are no government funds or national campaigns for rare diseases such as these. Let's face it: Had the Vorpahls not moved to Fort Atkinson, we quite possibly would never have heard of Niemann-Pick Disease-Type C. But they did, and we did, and the rest is history.

Of course, there is a long way to go after we all celebrate if — no, when — the FDA approves Zavesca for treating Niemann-Pick Disease. Keep in mind, though, that while exciting, this milestone writes just the newest chapter in a very lengthy book. In the meantime, the Vorpahls and all Niemann-Pick families continue to need our support.

Keep those donations and prayers coming.

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Since 1992, the National Niemann-Pick Foundation has raised \$4.3 million for education, family support, medical conferences and research, including that which led to the discovery of the NPC1 gene.

To help continue that work, send checks payable to the National Niemann-Pick Disease Foundation at: NNPFD, P.O. Box 310, Fort Atkinson, WI, 53538. Contributions also may be made on the foundation's website at www.nnpdf.org.

