

|Barb Vorpahl
FDA Presentation
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Good Afternoon. My name is Barb Vorpahl, and I am chair of the National Niemann-Pick Disease Foundation. My husband and Gary and I also had a daughter, Stacey who died at the age of 19 of Niemann-Pick Type C Disease. I want to thank you for this opportunity to speak to you today regarding your review of Zavesca (miglustat) as a treatment for NPC.

After Stacey was diagnosed at the age of two, our family joined together with other families to establish the National Niemann-Pick Disease Foundation. The foundation has been in existence for 18 years now, and I have served as a board member in all capacities over the years, including board chair for seven years.

I am here today as a representative of the 284 NPC families in our foundation's membership to express our firm belief in the value of Zavesca as a beneficial treatment for Niemann-Pick Type C Disease.

In the ten years since we first heard of Zavesca and it finally became available, 146 children and adults (that we know of) have died from NPC. 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.

When we first heard of a treatment with Zavesca we were hopeful. We were told it might help to slow the progression, but you can't imagine our excitement when we started hearing from parents that they were actually seeing improvements in the symptoms of this disease. Improvements in swallowing, speech, walking. We were overjoyed to hear that in siblings and others being treated with Zavesca early enough it was actually holding off the symptoms of this disease.

You have received testimonials from some of these families. (You have also heard some of the same findings this morning). You see the children in the audience....This drug is making a real difference to children and adultsit is giving them back some quality of life.

You have also heard from families like ours for whom it was too late for their children to benefit from Zavesca. What these letters don't tell you is the reality of this disease without any treatment.

With the diagnosis of NPC you are thrown into a world of the unknown. A world of wheelchairs, braces, feeding tubes, and trachs. Constant battles with insurance companies, medical specialists, school systems and social services.

Watching your child choke on each bite they take.

Sleepless nights suctioning your child so they won't suffocate in their own secretions.

Holding them close as you wait for another seizure to subside.

The fear of making your child laugh, so they won't collapse to the ground with cataplexy.

Seeing them stiffen again in pain but not being able to tell you what hurts.

As a parent, I know the feeling of helplessness watching your child deteriorate before your eyes... knowing there is nothing you can do to stop it.

ANYTHING THAT CAN INTERVENE WITH THIS RELENTLESS PROGRESSION IS ALL WE ASK FROM THIS COMMITTEE.

Over the past 18 years we have heard so many stories – the horrors of this disease and the devastation to the families affected by it. When their child is first diagnosed, parents contact us at the foundation, desperate for answers – for anything that can save their child. The hardest part was having to tell them there was nothing -- no treatment or cure –that we could offer them.

Since Zavesca became available this has changed. We finally have a beneficial drug. We finally have a glimmer of hope to offer these families. We can now offer them information on this medication and help them to get access “off label”.

Each year the foundation organizes a Family Support and Medical Conference where we bring NPD families together from all over the world. Through the years, I have come to know and love the families I have met.

Each year I have to steel myself for what I will see – how much ground the little ones have lost in the past year -- and to be there for the families whose child has died.

In the past few years this is starting to change, thanks to Zavesca. Many of the children and adults that have access to Zavesca are now at least “holding their own” against NPC. The progression of the disease isn't as aggressive as in the past. We believe Zavesca is making that difference.

But until Zavesca is approved, there are too many hurdles that stand in the way of children and adults who could benefit from this medication right now.

A negative recommendation from this committee would make getting Zavesca insurmountable for most families. The cost for the drug is around \$10,000 a month, or over \$100,000 a year. If Zavesca is not approved, we know insurance companies won't cover that cost. The majority of the families would not be able to afford it.

WITH ZAVESCA BEING THE ONLY PROMISING TREATMENT AVAILABLE AT THIS TIME IT WOULD BE A TRAGEDY IF EVEN ONE CHILD OR YOUNG ADULT IS NOT ABLE TO GET ZAVESCA!!

We know Zavesca is not a cure, but combined with other drugs on the horizon, Zavesca could serve as the core of a combination of therapies that could be an even more effective treatment for NPC.

(As we heard this morning)

A positive recommendation for Zavesca could also lead the way to early detection, diagnosis and intervention. It would move forward the establishment of the first International registry for NPC patients in the United States. This registry would help to find participants for future clinical trials.

A positive recommendation would continue the collection of information on this rare disorder.

All of these factors are extremely important in our desperate race against time.

On behalf of those facing a diagnosis of NPD Type C, I respectfully ask that you please consider carefully the data and the testimonials of the families who have been using Zavesca, and the positive impact it has had on their children's lives.

Zavesca came along too late for our daughter, Stacey, but with your decision we hope it won't be too late for the child or adult who is diagnosed with NPC today. Zavesca offers hope. Hope is a very powerful medicine.

Thank you.