

Re: NDA 21-438, ZAVESCA (miglustat) – Proposal for the treatment of Niemann-Pick Disease (type C)

Dear Ladies and Gentlemen:

The undersigned are parents of children stricken with Niemann-Pick type C disease (NPC); a devastating disease that steals the joys of childhood from seemingly normal children. We write to you in support of the application for ZAVESCA by Actelion Pharmaceuticals, Ltd. and the positive recommendation to approve ZAVESCA by the Endocrinologic and Metabolic Drugs Advisory Committee, asking you to help us save our children. In doing so, we want you to know how ZAVESCA has impacted the lives of our children, and how important ZAVESCA is to their futures.

Recognizing that a true cure for NPC (e.g. gene therapy) may be many years away, NPC families measure the success of any therapy in three key endpoints: delaying disease onset, slowing disease progression and extending life expectancy. We know that NPC patients on ZAVESCA for an extended period of time have experienced all three benefits, and the consistency of these benefits is far too universal to simply be anecdotal.

Clearly, delaying onset and slowing the progression of NPC have dramatic benefit for the quality of the lives of these patients, which for the majority of the patients who are children, is permitting them to enjoy a more normal childhood. With ZAVESCA, children's balance, gait, speech and swallowing are similar to a healthy child. The general weakness and pulmonary decline are held at bay. All of these benefits allow children on ZAVESCA extended years to run, play, laugh, perhaps ride a bike or play baseball far longer than would have been possible if they were not treated with ZAVESCA.

The greatest benefit of all is the extended longevity granted to these children by taking ZAVESCA. What can be more important to a parent than to have time with their child; extra time measured in years, not days, weeks or months; time that will get many of these afflicted children the benefit of further medical breakthroughs?

Further, these demonstrated benefits of ZAVESCA are widely accepted by NPC families as the first and only treatment for NPC. Relatively few families have insurance that pays for ZAVESCA, others suffer severe hardship, financial and personal to see that their child receives ZAVESCA, and still others cannot possibly afford the extraordinary cost and have to go without, facing an earlier death.

Without FDA approval for NPC, only a minority of those suffering from this disease has been able to afford ZAVESCA through insurance or with great hardship, and still many of those are unable to afford the optimum dosage. Therefore we tragically and unnecessarily continue to see too many children at young ages facing the ravages of NPC disease and losing their lives. The most fortunate children have been on ZAVESCA for many years and their disease progression has been slowed. And over the almost six years that NPC patients have been getting ZAVESCA, no NPC patients have experienced serious adverse effects.

Parents will continue to seek ZAVESCA for their children and the failure to approve ZAVESCA for the treatment of NPC will cause more families to suffer hardship and force even more children to go without. Given the fatality of NPC, the lack of any other treatment and the lack of any serious adverse effects, the interests of NPC patients can only be served by the approval of ZAVESCA for the treatment of NPC.

Please, give us more time with our children in support of our efforts to find ever more avenues to save their lives.

Sincerely,