

When Hope Is Gone: Saving Your Child

WASHINGTON (Ivanhoe Newswire) - What would you do if you found out your child had a terminal illness, or even worse, the one drug that could add precious years of life was denied by the FDA? Meet two families that are living this reality, and fighting to help other families like them



Dillon Papier is a small, but big time baseball fan. Behind his cap, Dillon's fighting an endless battle.

"We knew he had something wrong at two weeks," Mark Papier, Dillon's father, told Ivanhoe. "He had jaundice and he had a high liver enzyme count."

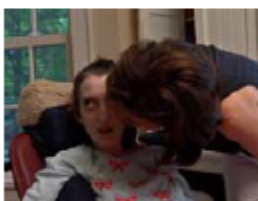
It took two years to diagnose him with Niemann-Pick Type C, a fatal genetic disease he shares with only 200 others in the U.S.

"We were in shock, in disbelief, and it was soul crushing," Darrile Papier, Dillon's mother, recalls



Niemann-Pick Type C causes muscle weakness, vision problems, seizures, dementia and other neurological symptoms that get worse over time. Most patients die within years of diagnosis. Dillon's only hope ... the drug Zavesca.

"A lot of the kids who didn't take the drug, they're already in wheel chairs and feeding tubes. I think our son is actually doing well," Mark said.



It's no cure, but Zavesca has been clinically proven to slow the progression of Niemann-Pick. It was approved in Europe last year, but, this March the FDA said they won't approve it until more studies are done. While the Papiers are fortunate their insurance company covers Zavesca off-label, many won't without FDA approval, leaving families alone to pick up the annual \$159,000 tab.

"You do everything you can as a parent to save your children," Andrea Marella said.

Philip and Andrea Marella say their children are living proof Zavesca works, and starting the drug early is crucial. Now 17, their daughter Dana began taking Zavesca when she was 11, and Andrew when he was five. He's now 10. By that age, Dana was already in a wheelchair.

"Andrew to this day, you wouldn't even know he has it," Andrea said. "He's seemingly normal. Dana on the other hand, might not be here today had she not been on Zavesca."

But Dana can no longer talk, she uses a feeding tube, needs daily breathing treatments and 'round the clock care. It's estimated only 25 percent of kids with Niemann-Pick Type C are on Zavesca.

"That's 75 percent of the kids that aren't benefiting from this drug," Philip said. "We would like to see all the children on the drug."

Both families continue to fight for Zavesca's approval, urging the FDA with letters.

"Every moment we are able to spend with our beloved son Dillon is a blessing," Darrile read an excerpt. "Every day that we are able to stage off the progression of this disease is a great day."

Both families are holding on to hope while they still can.

The Marella's insurance company also currently covers Zavesca off-label. They've created the



Dana's Angels Research Trust to raise money for Niemann-Pick Type C research. The Papiers also raise money through their organization Dillon's Fight For a Cure. To find out how you can help, visit <http://www.danasangels.org> and <http://www.dillonsfight.org>.

If you would like more information, please contact:

Dana's Angels Research Trust: <http://www.danasangels.org>
Dillon's Fight For a Cure: <http://www.dillonsfight.org>