

Urbana boy participates in drug trial for rare disorder

Researchers believe drug slows progression of neurological disease

By Morgan Young Staff Writer Thursday, February 28, 2013

When Dillon Papier of Urbana walked into Centerville Elementary School in Frederick on Monday, the 10-year-old was greeted with hugs and smiles from his fellow classmates and teachers.



“All the kids were excited to see him,” said Darrile Papier, Dillon’s mother. “They’re all thrilled to have him back.”

Dillon has Niemann-Pick disease type C, a rare neurological disorder which slowly degrades the nervous system.

There is no cure, and many children who suffer from the disease do not live past age 10, according to the National Niemann-Pick Disease Foundation.

Dillon was diagnosed when he was 3 years old, Papier said.

“We were in shock,” she said of the diagnosis. “It was soul crushing.”

Now, seven years later, Dillon is in fourth grade and has the chance help others like him.

For the last two weeks, Dillon has participated in a clinical trial at the National Institutes of Health in Bethesda for a drug that researchers hope will slow the progression of Niemann-Pick disease type C. He is one of nine patients chosen to be in the test group.

“We are so happy to have the Papier family as part of the [Niemann-Pick] research,” said Nicole Yanjanin, a NIH protocol coordinator for the trial. “Dillon has everyone in love with him.”

For about seven years, researchers have been preparing for the drug trial, headed by principal investigator Dr. Forbes D. Porter, by identifying the disease's biomarkers and conducting animal studies on the drug's effectiveness, Yanjanin said.

"We think based on animal studies that the earlier you treat the patient [with the drug] the higher the chances that you can prevent the progression [of the disease]," she said. "We're looking to see how the [biomarkers] change when someone gets the drug."

The trial is looking into optimal dosing of 2-hydroxypropyl-beta-cyclodextrin, which researchers believe helps to clear lipids that become trapped in the brain as a result of the disease, Yanjanin said.

Until now, people with Niemann-Pick type C were only treated for their symptoms, which can include slurred speech, difficulty swallowing and vertical gaze palsy, meaning patients have difficulty moving their eyes in up and down directions.

"We think that the drug can really have an impact on the cycle of the disease, and if we can do something to help our patients, we want to get there as soon as possible," Yanjanin said.



As part of the trial — which started a month ago — Dillon went through an hour-long surgery to place a Ommaya reservoir in his brain. The reservoir allows doctors to insert the drug directly into the brain. Each month for a year, Dillon will have some of his cerebrospinal fluid tested to see the effect of the drug dosages.

"For an unfortunate situation we are really fortunate," Papier said. "We're extremely hopeful."

As for Dillon, he won't be at school long.

On March 6, Dillon, who is an avid baseball fan, will travel with his family to Florida where they will join the Baltimore Orioles for their spring training.

During the visit, Dillon will spend time with his favorite player — second baseman Brian Roberts.

It will be the Papier family's seventh year going to Orioles' training.

"[The Orioles] called us up when Dillon was in the ICU," Papier said. "We're excited, [and] Dillon thoroughly enjoys [spring training]."