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GENERAL NEWS

Others Hit Unexpected Obstacles in NPC Survival Fight

Doctors say Niemann-Pick Type C disease can cause cirrhosis of the liver



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Dillon Papier, 12, peeks out of a room at the National Institutes of Health in December. Dillon was the first child enrolled in a clinical trial testing cyclodextrin as a potential therapy to treat Niemann-Pick Type C disease, a fatal cholesterol metabolism disorder. *LEXEY SWALL FOR THE WALL STREET JOURNAL*



Dillon Papier, 12 years old, was scheduled for another infusion of cyclodextrin in February in a clinical trial of the drug at the National Institutes of Health. But he landed in the emergency room with pains before he got the chance.

Tests found liver cancer, and doctors removed part of his liver on Feb. 19.

Dillon, who greets everyone with a high-five, kept telling his parents he was eager to get home and sleep in his own bed. Last Monday, he did.

Doctors say Niemann-Pick Type C disease can cause cirrhosis of the liver, a risk factor for liver cancer. Although the cancer is rare in people with NPC, the NIH will start checking patients in a long-term study.

Dillon, who was featured in a 2013 Wall Street Journal article on the disease, is one of 14 patients in the clinical trial of the drug that doctors hope will stop

or slow the progression of NPC.

There are more than a dozen children outside of the NIH trial also receiving cyclodextrin treatments. Some of the doctors attending these children are sharing the results with NIH and Vtesse, Inc., the Gaithersburg, Md.-based biotech that is sponsoring the drug trial.

Addison and Cassidy Hempel—11-year-old twins from Reno, Nev., who were also featured in the Journal series—were the first NPC patients in the U.S. to start taking cyclodextrin. Their parents, Chris and Hugh Hempel, received Food and Drug Administration approval in 2009 to give the experimental therapy to their children.

Addison and Cassidy experience seizures that have been difficult to control, according to Ms. Hempel and the girls' doctor, Caroline Hastings. The twins can't speak and need wheelchairs, but

their parents believe the drug is working. The girls remain alert and can eat and swallow. "They have a clarity in their eyes they didn't have," Ms. Hempel said. "They reach out to us."

A teacher comes to the house to work with the twins on assisted technology to help them communicate. Using a special computer, they are able to choose such favorite treats as Goldfish crackers, strawberries or pretzels by directing their gaze at the selection.

Their hearing is stable—hearing loss is a side effect of the treatment—but Ms. Hempel said she worries "every time we go for the hearing test."

Dillon Papier expects to start chemotherapy soon. When the doctors told his mother, Darrile Papier, that chemotherapy can cause hearing loss, she said, "I told them I already knew."

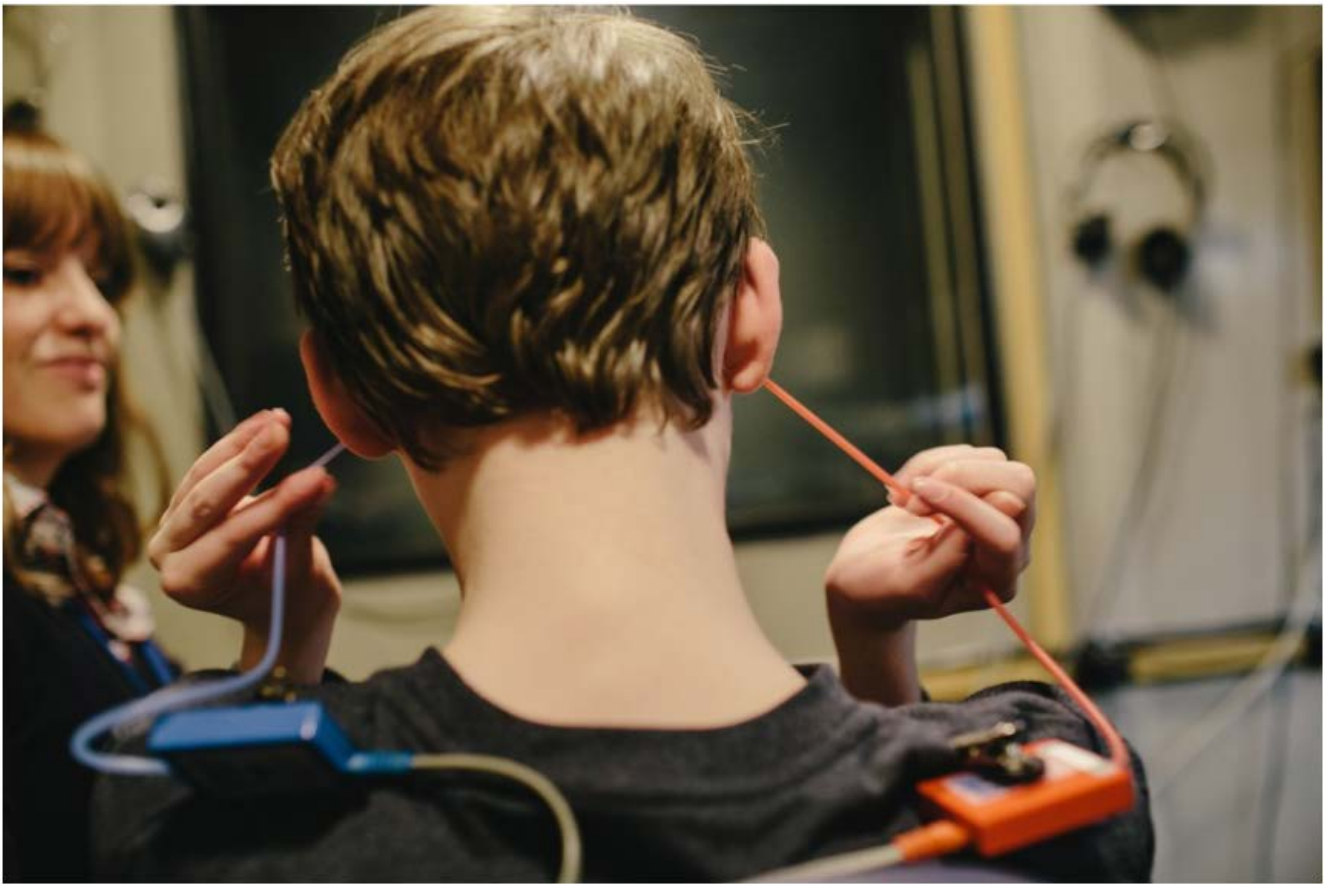
—Amy Dockser Marcus



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Dillon Papier, 12, has a hearing test administered by Kelly King at the NIH. Dillon experienced hearing loss after his previous infusion, a side effect of the drug, and his parents have struggled to decide whether to continue at the same dose. LEXEY SWALL FOR THE WALL STREET JOURNAL





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Dillon during his hearing test. *LEXEY SWALL FOR THE WALL STREET JOURNAL*



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Darrile Papier, right, talks to her son, Dillon, before he receives the cyclodextrin infusion. Dillon's parents decided to continue giving Dillon the higher dose, hoping the potential benefit will outweigh any further hearing loss. In late January, Dillon woke up in pain and was diagnosed with liver cancer. Dillon is expected to start chemotherapy treatments soon. When doctors talked to the Papiers about chemotherapy, they mentioned that sometimes the drugs can cause hearing loss. "I told them I already knew about that," Ms. Papier said. *LEXEY SWALL FOR THE WALL STREET JOURNAL*

