

CURRENTS

A Mom Brokers Treatment for Her Twins' Fatal Illness

Bucking Scientific Convention, Ms. Hempel Gets Researchers From Different Fields to Share Data on Potential Therapy

BY AMY DOCKSER MARCUS

From the moment her twin daughters, Addison and Cassidy, were diagnosed with a fatal genetic disease in October 2007, Chris Hempel has been searching for a drug that might save their lives.

The 5-year-old girls were diagnosed with a devastating cholesterol metabolism disorder known as Niemann-Pick Type C, which is ultimately fatal. Soon after, Ms. Hempel learned that researchers found that a form of a compound called cyclodextrin extended the lives of affected mice.

Ms. Hempel set out to gather as much data as possible. She got a list of all major cyclodextrin distributors and connected with one in Florida, who shared scientific studies and other information with her. She found a short report in the medical literature about a doctor who had treated a child with a different disease using cyclodextrin and tracked him down. She became increasingly hopeful that, although cyclodextrin isn't approved as a drug in the U.S., she might get the Food and Drug Administration to allow her to give cyclodextrin infusions to her girls as an experimental treatment.

Her search for information also led her to James Hildreth, 52, a pre-eminent AIDS researcher who heads the Center for AIDS Health Disparities Research at Meharry Medical College in Nashville, Tenn. It turned out that he too was seeking FDA approval to run a trial using cyclodextrin, in a vaginal cream to help prevent HIV transmission during heterosexual sex. Ms. Hempel wanted him to combine forces with the NP-C investigators to push forward cyclodextrin research.

That was only the beginning of Ms. Hempel's long journey through the health-care research community—a distributed and labyrinthine collection of researchers who, for all their expertise, often remain unaware of advances made elsewhere. The problem is even more acute among researchers working on different diseases. But for some serendipity, curiosity—or, in this case, a willful Ms. Hempel—some knowl-

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Drugs approved for one disease often turn out to be effective in others—frequently when someone has a hunch. Thalidomide, originally used for morning sickness but taken off the market because it caused birth defects, is being used in cancer treatment.

Researchers at Pfizer were developing Viagra to treat high blood pressure when they noticed during early tests that it treated impotence. But that happened within the same company. It is even more difficult when researchers are at different labs.

When Ms. Hempel, who lives in Reno, Nev., became passionate about Dr. Hildreth's work, she was determined to bridge the disparate knowledge. "Right now we have limited data on cyclodextrin. But what if a lot of people started looking at it from different angles and across different diseases?" Ms. Hempel said. "It could lead to something that helps save Addi and Cassi's lives."

Ms. Hempel had been researching cyclodextrin for months when she attended the June 2008 meeting in Tucson, Ariz., of the Ara Parseghian Medical Research Foundation, set up by the family of the legendary football coach who lost three grandchildren to NP-C disease. The foundation was providing some funding for cyclodextrin studies in the rare disease, and the latest data were presented there. In an email sent after the meeting, Ms. Hempel wrote to the NP-C researchers that, based on the data she heard, she and her husband, Hugh, planned to seek FDA approval to give the girls cyclodextrin infusions. "I feel very strongly that we must try this to help save Addi and Cassi from this horrible disease," she wrote.

She had already put together a three-inch binder of research studies about cyclodextrin. Working with three other families whose children have NP-C disease, they hired a scientist who began writing a request to the FDA for the Hempel children to receive cyclodextrin infusions. But Ms. Hempel knew that she needed more human data if



Chris Hempel, top with daughters Addison and Cassidy, has teamed with James Hildreth, bottom, and other researchers on a potential treatment for the twins.

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Ms. Hempel do research. He told her Dr. Hildreth was interested in the same compound for his work in HIV and suggested that the two of them talk.

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lieved the protein responsible for NP-C disease also plays an important role in HIV. And in previously published work, he showed that cyclodextrin appeared to inactivate the HIV virus and prevent it from replicating.

The talk galvanized Ms. Hempel. Dr. Hildreth offered to share what he knew about cyclodextrin's safety with the FDA in support of the Hempels' request. Ms. Hempel proposed that the two of them go to Johnson & Johnson, which had studied cyclodextrin, to see if the company would be interested in sponsoring a clinical trial. "I knew our stories would be even more powerful if we told them together," she said.

As is typical in the field, Dr. Hildreth was reluctant to share unpublished data, and he rarely went to scientific meetings that weren't related to HIV. He was moved by Ms. Hempel's efforts to help her children, but also surprised by her embrace of his work. "Some of the things we as scientists take for granted about how work will be done and the fact there are silos, with her there is none of that at all," he said.

When Ms. Hempel called a top National Institutes of Health AIDS researcher to tell him about Dr. Hildreth's findings and propose joint work in HIV and NP-C disease, Dr. Hildreth told her that a scientist never would have made such a call. In recent months, Ms. Hempel has introduced Dr. Hildreth to NP-C researchers who were also studying cyclodextrin. She also arranged for him to discuss his HIV findings with two Nobel Prize-winning scientists interested in Niemann-Pick proteins. "Our paths would not have crossed otherwise," he said.

He and Ms. Hempel recently had a conversation with senior officials at Johnson & Johnson. The FDA at first turned down the Hempels' request to do cyclodextrin infusions in the girls, concerned there wasn't enough human safety data. But after Ms. Hempel contacted them about her plight, the company wrote a letter to the FDA giving the agency permission to look at all of the safety data it had submitted related to cyclodextrin. The

FDA subsequently gave permission for the Hempels to proceed. The girls will start cyclodextrin infusions this month.

That might have been the end of the story except for Ms. Hempel's insistence that more was at stake, says Steven A. Silber, a vice president at Johnson & Johnson. After listening to Ms. Hempel and Dr. Hildreth's presentation, Dr. Silber set up a meeting so Dr. Hildreth can present his data to the head of one of its companies that makes anti-viral medications. Dr. Hildreth says that Ms. Hempel's involvement got his research "the attention of individuals higher up in the organization than I might have been able to get on my own."

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Parseghian, president of the foundation, says she hopes researchers working with cyclodextrin in other diseases will also attend. "We think there should be more cross-fertilization," she said. Dr. Hildreth says he plans to share his findings at the meeting.

Dr. Hildreth recognizes that his unusual partnership with Ms. Hempel also has some risks for the HIV trial he is planning. "It is a remote possibility, but is a possibility, that if her beautiful girls are done some harm by the infusions, that would clearly do harm to our efforts," he said. Still, he adds, "I spent a lot of time thinking about what I would do if I were in her position. My answer is I would do exactly the same thing."

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