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Gene therapy gives girl strength to fight disease

By Robert Miller THE NEWS-TIMES
NEW FAIRFIELD — A couple of weeks shy of her 7th birthday, Lindsay Karlin cannot say "Mama." But lately, she's been shaping her lips, as if to say the word.

She's also unclenching her hands and moving her arms and legs. A few months ago, her fingers, her limbs would either be almost rigid or floppy. She's doing certain things in a few seconds that, two or three months ago, would have taken twice as long. She's spontaneously making sounds. If she gets bumped, she cries; in May, she wouldn't have been aware she had been bumped.

"It's like she's a different person," said physical therapist Lori Melenski of Therapy Solutions.

Lindsay's parents, Helene and Dr. Roger Karlin, believe they know what's happened. An experimental gene therapy Lindsay received June 5 has kicked in, helping to offset the little girl's Canavan disease — the degenerative, fatal genetic disease she was born with.

To administer the therapy, Dr. Andrew Freese of Thomas Jefferson University Hospital in Philadelphia drilled six small holes in Lindsay's skull, inserted catheters and sent a solution with healthy, laboratory synthesized genes into her brain.

By now, Lindsay's hair has started to grow back, the incisions are almost healed and she's much more lively. "Something's going on," Helene Karlin said.

Max Randell, of Buffalo Creek, Ill., a Chicago suburb, had the same therapy in mid-June. His mother, Ilyce Randell, said she's seeing the same real improvements in her 3-year old son.

"It's not like he's getting up out of his wheelchair and walking," she said. "They're subtle changes. But he's doing things like looking you straight in the eye — before he'd just look in the general area of your face. Everyone who works with him has seen it."

The work to treat children with Canavan disease through gene therapy is gaining national attention; the American Museum of Natural History in New York City features the disease in its year-long exhibit on the human genome.

But the Karlins' and the Randells' joy in seeing their children improve even a bit is mixed with anger and uncertainty over how next to proceed. The gene therapy the two received in June was ready to be used in the spring of 2000. But fears of litigation against research institutions and doctors shifted the process into slow motion.

"And in that year, Lindsay's brain suffered some atrophy," Helene Karlin said. "Her eyes lost the ability to track objects. And she was starting to be out of it. Now she's back. She's awake and aware. But I can't let her degenerate again."

2001-07-03



The News-Times/Carol Kaliff

Helene Karlin of New Fairfield cradles daughter Lindsay, who suffers from Canavan disease, a rare genetic disease that occurs most often in families where both parents are Ashkenazi Jews.

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Canavan disease is a rare genetic disease that occurs most often in families where both parents are Ashkenazi Jews.

Because of the mutation of a single gene, Canavan sufferers have a buildup of N-acetylaspartate acid, or NAA, in the brain. This prevents them from growing myelin, the fatty sheath that normally insulates nerve cells and allow the cells to carry messages.

Because those messages never get sent, most Canavan children cannot walk, talk, sit or feed themselves. As the disease progresses, they become blind and paralyzed. Most don't live past age 10.

When Lindsay was diagnosed with Canavan disease in 1995, doctors told the Karlins there was nothing they could do. Refusing to passively accept a death sentence, they spent a year attempting to learn everything they could about the disease, eventually founding the Canavan Research Foundation.

Their work led them to Dr. Matthew During, a New Zealand-born doctor studying at Yale University School of Medicine. During proposed treating children with Canavan disease by injecting healthy genes, synthesized in a laboratory, into their brains.

From 1996 to 1999, Lindsay underwent this procedure three times. Each time she improved — she's the first child to grow myelin because of gene therapy. But after the improvements, she slowly began to regress.

The researchers working on Canavan disease decided what they needed was a better way to spread the healthy genes more completely into the brain. They chose a virus that can reach about 100 million brain cells, compared to 100,000 in the solutions used in the first three trials.

But in 1999, an 18-year old male died at the University of Pennsylvania while undergoing gene therapy for a rare liver disease. In the aftermath, federal agencies approving gene therapy and the universities carrying out research became cautious about letting experimental work proceed.

While such caution may have been prudent to university deans fearing lawsuits, it was intolerable to parents watching their children languish with a progressive disease.

Eventually, the parents prevailed. Now that this round of gene therapy for Canavan disease is under way, another six children will receive it and there are another 10 who want it.

The Canavan Research Foundation, is committed to keeping this research going, despite bureaucratic slowdowns. There is also the belief that stem cell research will be an even more efficient way to repair the damage of malfunctioning genes.

But between battles, the Karlins and the Randells also have a profound appreciation for the therapy that's keeping their children alive.

"I didn't want to get my hopes up," Ilyce Randell said. "But looking at it objectively, the changes are amazing."

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