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**LOCAL**

2004-08-10

**Stem cell policy sparks hope, fury**  
**Parents see progress in daughter's fight with Canavan disease**

By Robert Miller

*THE NEWS-TIMES*

At rest, Lindsay Karlin has a hard time making a sound. Her chest muscles and diaphragm are so weak it's hard for her to work the wind up to her larynx.

But her parents, Dr. Roger and Helene Karlin, have been steadfast in getting her to respond with her voice, not just her hands. It may take her a minute — her sweet face, her small, wheelchair-bound body pushing hard — but then a small murmur gets out. And everyone is happy.

"She's here, cognitively," Helene Karlin said. "She understands."

And that, in itself, is a miracle. Lindsay cannot walk, or talk, or feed herself. Even lifting her arm is an effort. Her vision is poor. It takes her some effort to hold her head erect.

But Lindsay, at 10 years old, has beaten the odds. Most children with the genetic illness she suffers from — Canavan disease — are paralyzed, blind, largely lost to the world by age 5. Lindsay is in the world. "She's the only Canavan child we know that doesn't have a gastrointestinal tube for feeding," Helene Karlin said.



*The News-Times/Carol Kaliff*

Canavan disease hasn't stopped Lindsay Karlin from playing a keyboard piano.

feel like we've stabilized the disease," Helene said. "We're not talking about a degenerative progression. We see improvements. We can enjoy the days passing."

But this success comes at a time when the future of Canavan disease research is in limbo.



*The News-Times/Carol Kaliff*

Lindsay Karlin, 10, with her father, Dr. Roger Karlin. Lindsay suffers from Canavan disease, a genetic illness that leaves many victims paralyzed and blind.

"That's so important," said Dr. Paola Leone, the researcher who has worked with the Karlins for much of Lindsay's life. "It means her brain is still signaling to swallow."

Thanks to a decade of groundbreaking research on Canavan disease — pushed to a large extent by the Karlins — Lindsay's life has been stabilized. Injecting healthy genes into her brain has stabilized her condition for the past three years. New drug therapies have helped as well.

The Karlins no longer hold out hope Lindsay will someday walk and run and talk like other 10-year-olds. But they are profoundly grateful they can go to sleep without worrying their daughter will die in the middle of the night from a seizure.

"We really

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Parents of Canavan children have raised funds to pay for medical treatment and research. The Canavan Research Foundation held its annual fund-raiser at the Ridgewood Country Club in Danbury on Monday.

But the amount the families can raise is limited. And it's unclear whether the National Institutes of Health — which gave \$2.1 million for Canavan research to fund the gene therapy program — will renew its funding when the grant period ends in mid-2005.

"I've been in this business for 18 years and I've learned always to be positive, to hope," said Leone, who heads the Canavan research project at the University of Medicine and Dentistry at New Jersey/Robert Wood Johnson Medical School in Camden, N.J. "But if we don't get the funding, we'll have to dissolve the lab. It would be heartbreaking."



*The News-Times/Carol Kaliff*

Advocates say stem cell research could help make life easier for people like Lindsay Karlin, 10, who suffers from Canavan disease.

At the same time, Leone, the Karlins, and other Canavan parents are pushing ahead to advance work on stem cell research on Canavan disease. This research holds enormous potential, doctors and Canavan advocates say.

Stem cells are the first cells that develop out of a fertilized egg. Because they eventually differentiate themselves to produce all of a body's organs, researchers now hope they may also have the power to regenerate into any organ — nerves, muscles, brain tissue, skin — where they are placed, repairing injured organs and providing healthy tissue.

But stem cell research is also so controversial in the United States that President Bush has put limits on it. People in the pro-life movement object to the use of aborted fetal tissue in stem cell research. Others worry the use of stem cells may someday be used in an attempt at human cloning.

In 2001, Bush decided the government position on stem cells would be to promote research into adult stem cells — cells in adults that help specific organs regenerate — while limiting research on embryonic stem cells to a set number of established lines. By doing so, the Bush administration allowed stem cell research to go forward while making it clear it would not allow new embryos or aborted fetal tissue to be used for research.

The Bush administration approved \$190 million in research funds for adult stem cells in 2003, and \$24.8 million for embryonic stem cell research.

Researchers say stem cells could hold keys to breakthrough treatment of a number of diseases, including Alzheimer's.

Karlin said he found some irony in Ron Reagan's address to the Democratic National Convention urging the nation to support stem cell research. When Reagan's father, Ronald Reagan, was president, Karlin said, the federal bureaucracy paid little attention to the possibilities of stem cell research.

"Then, after he left the presidency, he developed Alzheimer's," Karlin said. "I was pleased to see his son being so pro-stem cell. But I think it's a shame that it takes a family experience to understand that there are a variety of people out there that need this help. Then again, I'm not sure we would either, if it wasn't for Lindsay."

Lindsay was an infant when her parents realized she was not responding to the world the way a healthy infant should. Testing showed Lindsay had Canavan disease, a rare genetic condition that occurs only when both parents have faulty genes that link up in the child.

Canavan belongs to a family of diseases called leukodystrophies. In them, a genetic breakdown stops the production of myelin, the white, fatty lining that sheaths nerve cells and permits them to carry messages in and from the brain.

Most Canavan children begin to deteriorate into blindness, seizures and paralysis as young children and seldom live to be 10 years old.

Instead of yielding to the inevitable, the Karlins and their doctors came up with a plan — produce healthy, synthetic genes in the laboratory, then inject them directly into the brains of Canavan patients.

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Lindsay has had four rounds of gene therapy. After the first three, she would improve — she was the first child to grow myelin because of gene therapy. But within a few months, she would slowly decline. The breakthrough treatment came in 2001, when Leone and other physicians devised a much better delivery system for the gene, using a viral vector that spread the healthy gene throughout the entire brain rather than just at the injection sites.

Roger Karlin said Lindsay's latest MRI again shows six additional sites where myelin is growing. But he said the brain may need a lot more myelin to function properly.

Along with Lindsay, nine other children with Canavan disease have had gene therapy. The Karlins said all have shown improved cognition, a halt in their physical decline and myelin regeneration.

Max Randell of Buffalo Springs, Ill., is one of the shining examples of the success of gene therapy. He began receiving gene therapy at 11 months. In October, he'll be 7. While he can't talk, he can recognize letters and words, spell, read and write, thanks to a computer.

"He's actually pretty close to where he should be intellectually for a 6-year-old," his mother, Ilyce, said.

More remarkably, using a joystick, he can pilot his motorized wheelchair on his own — a first for a Canavan child. "The wheelchair came with four motorized settings, with one being the slowest — the learner's setting," Ilyce Randell said. "Max hated that. He wanted it up at four."

The gene therapy research for Canavan disease has had ramifications in other research. There are now two ongoing gene therapy trials — one for Parkinson's disease, the other for Batten disease, a rare neurological disorder — that are both using the viral vector and the delivery stem first devised for the Canavan trials.

"It's taking them a lot less work and a lot less money to do this because we did it first," Leone said. "They know it's safe now."

Leone is now completing her reports on the first Canavan trials. She believes the end result will prove conclusively that children who received gene therapy had neurological improvements because of it.

The best outcome of this would be for Leone's lab to receive a second round of NIH funding, allowing her to start a Phase Two trial that could treat more children with Canavan disease.

"Because of the work I've done, I get e-mails from everywhere in the world about children with Canavan disease," she said.

While waiting for word on the funding, the researchers are thinking about the next step forward in treating Canavan children: stem cell therapy. Leone said she already has collaborators in Italy who have begun initial work in this field.

"Scientists now believe it's the most promising field of biomedical research," said Eve Harold, public education manager for the Stem Cell Research Foundation in Clarksburg, Md. In the laboratory, she said, stem cells have transformed themselves as into skin, retinal tissues, cartilage and kidney tissue. There are indications stem cells can repair damaged heart tissue and spinal column injuries.

Because of the complexities of Canavan disease, children with it would likely need a two-pronged treatment. First, a child would need gene therapy to clear the brain of a compound called N-acetylaspartate acid or NAA. While NAA is essential for producing myelin, in Canavan's patients the compound accumulates in the brain at toxic levels.

Once Canavan patients receive treatment to remove excess NAA, stem cells would be implanted to repair any damage caused by the disease.

The U.S. government has approved 21 stem cells lines for use, but Harold said currently the number available to researchers is between 15 to 19 lines.

"It's not a lot at all," she said. "They don't have the genetic diversity researchers need."

Karlin and others say the Bush administration policy lets many stem cells that could be used for research fall by the wayside. For women who use in vitro fertilization to become pregnant, there are often several fertilized eggs that are not transplanted back into the uterus. These are often frozen — for later use in case the pregnancy fails. They can get thrown away if not used.

"Abortion is legal in this country," Karlin said. "Aborted fetuses are just thrown away. Even if you

believe that life begins at conception, why not use this fetal tissue to help people with a variety of disease? Why not help mankind?"

The Karlins admit they are adjusting their expectations for their daughter. She may never walk or speak. But Helene Karlin said, it's possible she could communicate with the world through a computer she activates with her mouth. It's even possible she could use a motorized wheelchair, given the chance.

The Karlins look at the promise of stem cell research, and they look at the smiling face of their daughter, and they see miracles waiting. "Lindsay has potential," Helene Karlin said. "This could help her."

Contact Robert Miller

at [bmiller@newstimes.com](mailto:bmiller@newstimes.com)

or at (203) 731-3345.

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