

New genes provide hope to Palatine boy

By Ruth Gesmer Silverman Daily Herald Correspondent

Posted on July 05, 2001



Only days after undergoing a groundbreaking experimental gene transplant, 31/2-year-old Max Randell was cradled in his mother's arms on the floor of their Palatine condo, listening to her croon a little song. "Who has 90 billion new genes in his head?" Ilyce Randell sang.

Oblivious to his shaved head and the six incisions over the healing holes in his skull, Max responded by deliberately blinking his thick-fringed blue eyes. The song over, Ilyce shifted him from one arm to the other and called, "Max." He looked intently at his mother and reached out to pat her cheek. Eyes blink, hand pats. These are good signs.

Max has Canavan disease, meaning he is missing the gene that produces myelin, a protective sheath around the nerves in the brain. Without myelin, the brain turns into a spongy, water-filled mass.

Children with Canavan disease are profoundly disabled and usually die by the age of 10. But Max has a little hope now. He was among a group of Canavan children who underwent a six-hour experimental procedure in Philadelphia at Thomas Jefferson University Hospital about two weeks ago.

Ilyce and Mike Randell say it's too early to tell if their son has benefited from the procedure. Ilyce said she thinks Max is already more focused.

Their occupational therapist, Debbie Solomon, has seen some small signs of progress. "He was able to hold himself up on his arms quite well when I placed him on his tummy," Solomon said.

Researcher Dr. Paola Leone developed the gene therapy and the pump that delivers it. With NBC "Dateline" cameras rolling for a one-hour program on Canavan disease and Max to be broadcast this fall, Leone pushed the button on the pump to introduce the 0.9 cc's of gene-laden liquid into six sites in his brain. "All I could think of while I was doing it was 'I can't wait for it to be over so I can tell the family that everything is fine,'" Leone said later.

Among those waiting with baited breath was Ilyce's mother, Peggy Shapiro-Nyeholt of Des Plaines. "I would walk over tacks for the rest of my life to save him," she said. "When he stays over here ... he wakes up smiling and loves each day; he is the purest, sweetest little soul, and he clearly loves life.

"In the middle of making sure Max's recovery goes smoothly, Ilyce and Mike are packing for their move to Buffalo Grove, where the federally-registered Canavan Research Organization of Illinois, based in their home, will be located after July 9.

They are also planning several fund-raisers in the next few weeks for the foundation, which helps pay for research, including Leone's investigations into stem-cell therapy. The foundation also directs money toward a possible pharmacological approach to treatment.

Money also is used to increase public awareness on research and therapy to help those who suffer from other genetic disorders, such as Parkinson's, multiple sclerosis, Tay Sachs and Alzheimer's. "It's not just about funding research for Canavan disease," said Ilyce Randell.

Their big fund-raiser is planned for Sept. 22, at Watercress Banquets in Palatine, where they are hoping to raise at least \$30,000.

For information on the disease and research, call (800) 833-2194 or see www.canavanresearch.org.

Please note - This promising Canavan Gene Therapy Trial (described in the article above) is currently at Cooper Health System, a division of Robert Wood Johnson Hospitals, Camden, N.J.