Gene Therapy Shows Promise for Brain Disorder

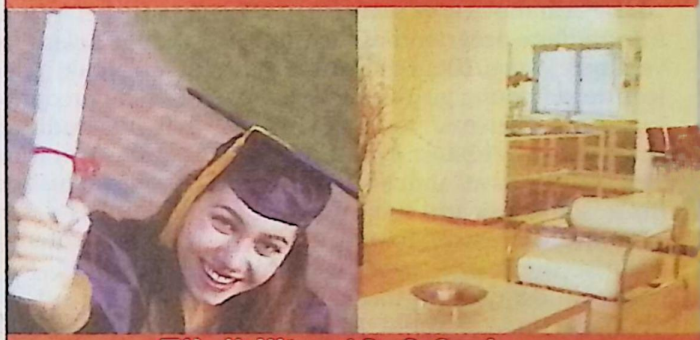
THE children all have late infantile neuronal ceroid lipofuscinosis (LINCL), a form of the neurodegenerative disorder Batten disease. They were born without a working copy of CLN2, a gene whose protein helps lysosomes--the cell's garbage-disposing structures--break down a waste product called lipofuscin. As a result, lipofuscin builds up and eventually destroy neurons, causing the brain to shrink. Children with LINCL seem normal at birth but by age 2 to 4 show signs of developmental problems and often have seizures. Eventually blind and confined to a wheelchair, they usually die by 8 to 12 years of age.

A few years ago, gene therapy researcher Ronald Crystal and colleagues at Weill Cornell Medical College in New York City successfully slowed LINCL in mice using gene therapy in the brain. To test the safety of the approach in humans, the team treated 10 LINCL patients ranging in age from 3 to 10 years, starting in 2004. After anesthetizing the children, the researchers drilled six 2-mm-wide holes in their skulls. They then dripped in a solution of a harmless virus that had been modified to carry a good copy of the CLN2 gene. Four children had an immune response, but it was mild. One patient developed seizures 2 weeks later and died 49 days after the surgery. However, she did not have brain inflammation, and Crystal says it was not clear whether her death had anything to do with the gene therapy.

Over the next 18 months, Crystal's team conducted neurological tests and magnetic resonance imaging (MRI) scans. Although the MRI scans suggested that the brains of treated children were shrinking less than they would have otherwise, the results were not statistically significant. On measures of motor function, language, and seizure frequency, however, the children appeared to deteriorate more slowly compared with untreated patients, dropping about two points less on a nine-point scale than controls. "This is suggestive [of efficacy] and encourages us to move on," says Crystal, whose team reports its results online today in Human Gene Therapy. The roughly $8 million study was funded by Nathan's Battle Foundation, which was started by the parents of two siblings with LINCL, both of whom were enrolled in the study.

Source: sciencemag.org

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