Experimental gene therapy allows kids with inherited deafness to hear

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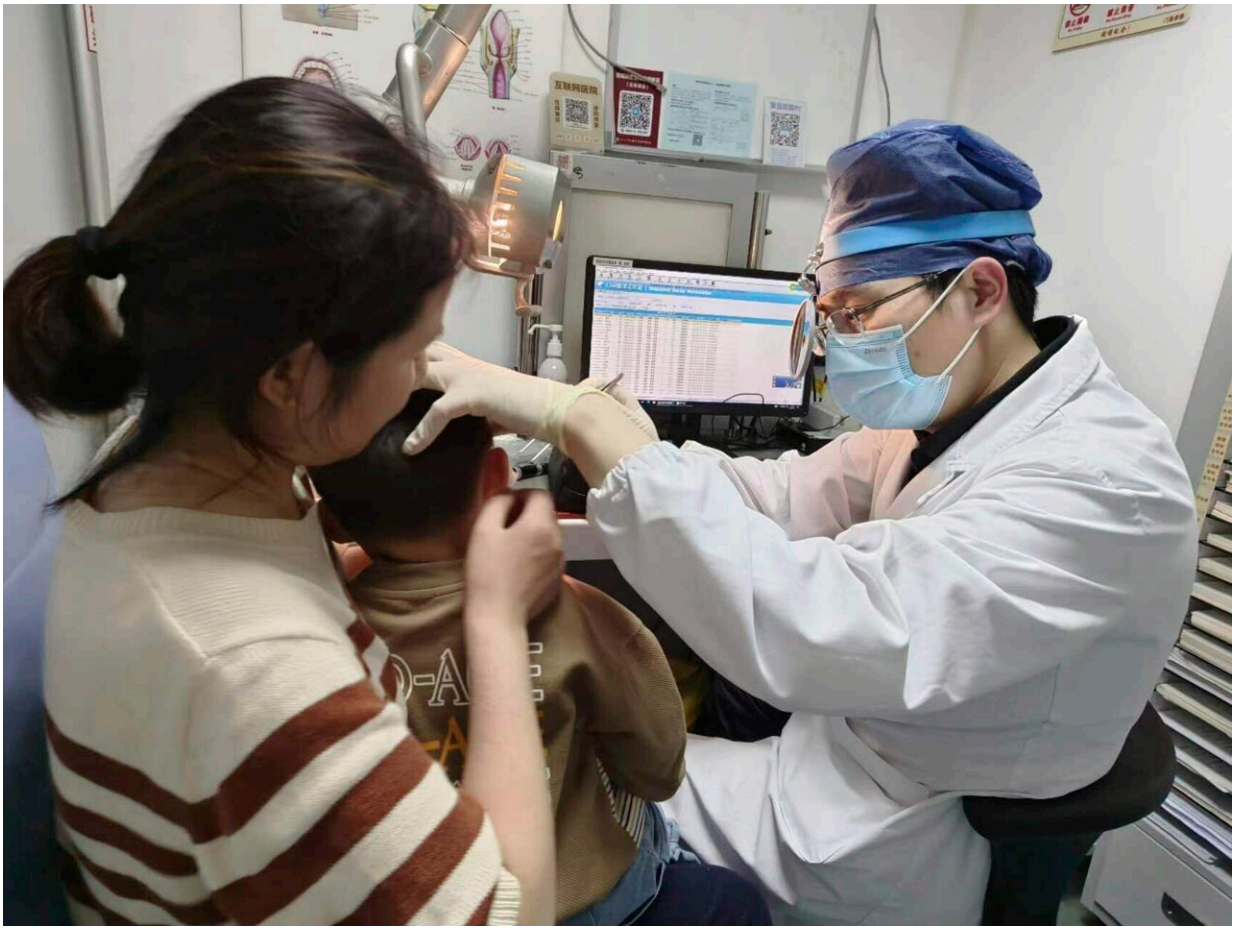

In this photo provided by researchers in January 2024, Dr. Yilai Shu examines a young patient at the Eye & ENT Hospital of Fudan University in Shanghai, China, after a gene therapy procedure for hereditary deafness. A small study published Wednesday, Jan. 25, 2024, in the journal Lancet, documents significantly restored hearing in five of six kids treated in China. Credit: Dr. Yilai Shu via AP

Gene therapy has allowed several children born with inherited deafness to hear.

A small study published Wednesday documents significantly restored hearing in five of six kids treated in China. On Tuesday, the Children's Hospital of Philadelphia announced similar improvements in an 11-year-old boy treated there. And earlier this month, Chinese researchers published a study showing much the same in two other children.

So far, the experimental therapies target only one rare condition. But scientists say similar treatments could someday help many more kids with other types of deafness caused by genes. Globally, 34 million children have deafness or hearing loss, and genes are responsible for up to 60% of cases. Hereditary deafness is the latest condition scientists are targeting with gene therapy, which is already approved to treat illnesses such as sickle cell disease and severe hemophilia.

Children with hereditary deafness often get a device called a cochlear implant that helps them hear sound.

"No treatment could reverse hearing loss … That's why we were always trying to develop a therapy," said Zheng-Yi Chen of Boston's Mass Eye and Ear, a senior author of the study published Wednesday in the journal Lancet. "We couldn't be more happy or excited about the results."

The team captured patients' progress in videos. One shows a baby, who previously couldn't hear at all, looking back in response to a doctor's words six weeks after treatment. Another shows a little girl 13 weeks after treatment repeating father, mother, grandmother, sister and "I love you."

In this image from video provided by the Children's Hospital of Philadelphia, an 11-year-old boy who was born with hereditary deafness prepares for a gene therapy procedure in Philadelphia in October 2023. On Tuesday, Jan. 23, 2024, the hospital announced his hearing has improved enough that he now has only mild to moderate hearing loss in the ear that was treated, and is hearing sound for the first time in his life. Credit: Children's Hospital of Philadelphia via AP

All the children in the experiments have a condition that accounts for 2% to 8% of inherited deafness. It's caused by mutations in a gene responsible for an inner ear protein called otoferlin, which helps hair cells transmit sound to the brain. The one-time therapy delivers a functional copy of that gene to the inner ear during a surgical procedure. Most of the kids were treated in one ear, although one child in the two-person study was treated in both ears.

The study with six children took place at Fudan University in Shanghai, co-led by Dr. Yilai Shu, who trained in Chen's lab, which collaborated on the research. Funders include Chinese science organizations and biotech company Shanghai Refreshgene Therapeutics.

Researchers observed the children for about six months. They don't know why the treatment didn't work in one of them. But the five others, who previously had complete deafness, can now hear a regular conversation and talk with others. Chen estimates they now hear at a level around 60% to 70% of normal. The therapy caused no major side effects.

Preliminary results from other research have been just as positive. New York's Regeneron Pharmaceuticals announced in October that a child under 2 in a study they sponsored with Decibel Therapeutics showed improvements six weeks after gene therapy. The Philadelphia hospital—one of several sites in a test sponsored by a subsidiary of Eli Lilly called Akouos—reported that their patient, Aissam Dam of Spain, heard sounds for the first time after being treated in October. Though they are muffled like he's wearing foam earplugs, he's now able to hear his father's voice and cars on the road, said Dr. John Germiller, who led the research in Philadelphia.

"It was a dramatic improvement," Germiller said. "His hearing is improved from a state of complete and profound deafness with no sound at all to the level of mild to moderate hearing loss, which you can say is a mild disability. And that's very exciting for us and for everyone. "

Columbia University's Dr. Lawrence Lustig, who is involved in the Regeneron trial, said although the children in these studies don't wind up with perfect hearing, "even a moderate hearing loss recovery in these kids is pretty astounding."

In this image from video provided by the Children's Hospital of Philadelphia, an 11-year-old boy who was born with hereditary deafness is given a hearing test in Philadelphia, after he underwent a gene therapy procedure in October 2023. On Tuesday, Jan. 23, 2024, the hospital announced his hearing has improved enough that he now has only mild to moderate hearing loss in the ear that was treated, and is hearing sound for the first time in his life. Credit: Children's Hospital of Philadelphia via AP

Still, he added, many questions remain, such as how long the therapies will last and whether hearing will continue to improve in the kids.

Also, some people consider gene therapy for deafness ethically problematic. Teresa Blankmeyer Burke, a deaf philosophy professor and bioethicist at Gallaudet University, said in an email that there's no consensus about the need for gene therapy targeting deafness. She also

pointed out that deafness doesn't cause severe or deadly illness like, for example, sickle cell disease. She said it's important to engage with deaf community members about prioritization of gene therapy, "particularly as this is perceived by many as potentially an existential threat to the flourishing of signing Deaf communities."

Meanwhile, researchers said their work is moving forward.

"This is real proof showing gene therapy is working," Chen said. "It opens up the whole field."

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