Gene therapy treatment restores hearing to five children in China. Will the results last?

Yiyi [Li Xincheng] is one of several deaf children who scientists in China say are the first people ever to have their natural hearing pathway restored in a dramatic new demonstration of the possibilities of gene therapy. The feat is even more remarkable because until now, no drug of any kind has ever been able to improve hearing.

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The new treatment will not help everyone who is deaf. It applies only to one specific cause of deafness at birth: a defect in a gene that produces a protein called otoferlin. The inner ear contains about 16,000 hair cells, so called because they have comblike extensions that vibrate to different frequencies of sound. Without otoferlin, these cells can’t transmit the chemicals that relay information to the brain.

Auditory hair cells respond relatively well to gene therapy, easily taking up new DNA. And they don’t grow or get replaced during a person’s life. This is a reason why very loud noises can lead to permanent hearing loss: they can kill the hair cells. But it also means that if a replacement gene is added to the cells, it could remain active for a lifetime, although [surgeon Yilai] Shu cautions it’s unknown how long the effect will persist.

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