

FDA approves first gene therapy for genetic hearing loss

In an unusual move, the drugmaker Regeneron is making the treatment available for free to patients in the United States.



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— The FDA approved a gene-therapy drug from Regeneron to treat genetic hearing loss.

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By Berkeley Lovelace Jr.

The Food and Drug Administration on Thursday [approved](#) Regeneron's Otarmeni, the first [gene therapy](#) for genetic hearing loss.



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The drugmaker said it will offer the drug for free to U.S. patients.

Otarmeni is approved to treat a very rare form of hearing loss that affects about 50 babies born in the U.S. each year and is caused by a mutation in a gene called OTOF. The approval was granted under the [Commissioner's National Priority Voucher](#), an FDA pilot program intended to fast-track drug reviews.

"This really is life-changing for families with children with hearing loss," Dr. Eliot Shearer, a pediatric otolaryngologist at Boston Children's Hospital and a principal investigator on the Regeneron trial.

The only other treatment option for children with genetic deafness is cochlear implants, Shearer said, which restore the ability to hear speech and music, but reduce the fidelity of the sound.

With gene therapy, the improved hearing is "on 24/7 and doesn't rely on batteries," he added.

The drug works by replacing the faulty OTOF gene, which is supposed to work by giving the body instructions to make a protein called otoferlin. This protein plays a role in transmitting pulses from cochlear cells within the ear to nearby nerves, which carry the signals on to the brain. Without otoferlin, sound never reaches the brain.

Thursday's approval was based on a trial of 20 children diagnosed with the OTOF mutation who got a single dose of the therapy in one or both ears.

The trial found that it improved hearing in 16 of the 20 children, with five kids developing the ability to detect whispers. Side effects included infection or inflammation of the middle ear, vomiting, nausea and dizziness.

The approval was touted by President Donald Trump during an Oval Office announcement Thursday afternoon about a drug pricing deal with Regeneron to provide many of its drugs at a discount [on the self-pay platform TrumpRx](#).



— President Donald Trump announced a deal with Regeneron in the Oval Office on Thursday.

Alex Wong / Getty Images

While the company is providing the drug at no cost, it has no say over the cost of administering the drug.

Shearer said the procedure is similar to surgery for cochlear implants, where doctors access the inner ear to deliver the drug while the patient is under general anesthesia.

The drug is not approved outside the U.S., so Regeneron does not have a list price for people abroad who want to get the treatment. Gene therapy prices can be in the millions.

Sarah Emond, president and CEO of the Institute for Clinical and Economic Review, a nonprofit group that evaluates the cost and effectiveness of drugs, said Regeneron should be commended on its pricing.

“Regeneron has shown us that one option we can consider that will ensure affordable access for patients to these therapies is to not charge the health system for the therapy,” Emond said.



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