The Food and Drug Administration, thanks to the Commissioner's National Priority Voucher for expediating drug approval analyses, recently approved the first gene therapy drug, called Otarmeni from Regeneron, for hereditary hearing loss. With this new approval, Regeneron will apparently provide the drug for free to the patients in need. The specific hearing impairment Otarmeni can treat is rare, as it only affects around 50 newborns yearly in the United States. A mutation in a gene called OTOF causes the hearing loss (1).

The only treatment available for patients before this approval was the cochlear implant, which only allows the patients to hear speech and music at a lower accuracy of which the sound is reproduced. Otarmeni functions by substituting for the defective OTOF gene that causes the hearing impairment. According to the NBC News article, the OTOF gene's role, under normal conditions, is to provide the body instructions to produce the protein otoferlin. Otoferlin transmits pulses from the cochlear cell in the ear to adjacent nerves that carry the signals to the brain (1).

The FDA's approval of the drug was established on the success of a trial performed on 20 children with the mutation in their OTOF gene. The children received a dose of Otarmeni in one or two of their ears. Out of the 20 children, 16 of them had their hearing improve and 5 of those could hear whispers. Otarmeni can cause side effects such as infection or inflammation in the middle ear, dizziness, nausea, or vomiting. The procedure for administering the drug would be like that for the cochlear implants with the patient under general anesthesia (1).

During the approval, President Donald Trump announced that there would be a price discount for Otarmeni through TrumpRx. Even though Regeneron will be providing the drug for free, the administration of the drug may still have to be paid. Regeneron also does not have a price for patients who would come from abroad since the drug is not approved in any other country (1).

The NBC news article correctly explains the nature of the genetic hearing loss, and the general outcome of the drug use. However, it does oversimplify the biology and physiology of the hearing disorder and gene therapy for it. The news article summarizes that the drug trial was very successful without including the variability in results, the limitations of the drug, and the constraints of eligibility for the drug. Therefore, the news article creates a misconception that by just replacing the defective gene, hearing is restored (2,3).

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