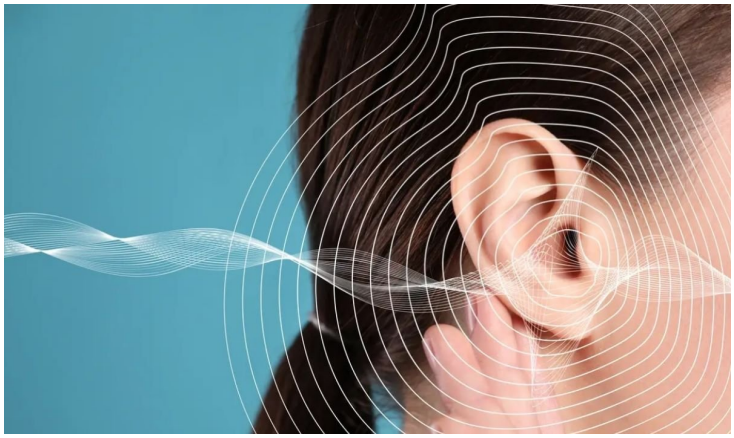
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US FDA approves first-ever gene therapy for treatment of genetic hearing loss

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Potential treatment for patients with OTOF gene-associated severe-to-profound and profound hearing loss



The US Food and Drug Administration (FDA) has approved Otarmeni (lunsotogene parvec-cwha), the first-ever dual adeno-associated virus (AAV) vector-based gene therapy. Otarmeni is indicated for the treatment of pediatric and adult patients with severe-to-profound and profound sensorineural hearing loss (any frequency >90 dB HL) associated with molecularly confirmed biallelic variants in the *OTOF* gene. US FDA has granted accelerated approval of Otarmeni to Regeneron Pharmaceuticals, Inc.

Prior to this approval, no disease modifying treatments existed for *OTOF*-related deafness. Otarmeni is for patients with preserved outer hair cell function and no prior cochlear implant in the same ear.

Genetic mutations cause about half of congenital hearing loss. Variants in the *OTOF* gene account for 2% to 8% of inherited, non-syndromic cases. Patients with two nonworking copies do not produce otoferlin, disrupting sound signal transmission. Delayed diagnosis can lead to missed treatment windows and lasting speech and language delays.

Otarmeni and the administration kit are a one time biologic-device combination product. It includes a dual adeno-associated virus serotype 1 (AAV1) vector gene therapy administered as a single dose per ear surgically into the cochlea via a syringe and catheter provided in the Administration Kit and connected to an infusion pump. Otarmeni delivers a functional copy of the *OTOF* gene to inner hair cells to restore otoferlin production and auditory signaling.