CARECONTINUUM™

UTILIZATION MANAGEMENT MEDICAL POLICY

POLICY: Otolaryngology – Gene Therapy – Otarmeni Utilization Management Medical Policy
- Otarmeni™ (lunsotogene parvec-cwha intracochlear infusion – Regeneron)

REVIEW DATE: 09/23/2026

OVERVIEW

Otarmeni, an adeno-associated virus vector-based gene therapy, is indicated for the treatment of pediatric patients and adults with **severe-to-profound and profound sensorineural hearing loss (any frequency > 90 decibel hearing level [dB HL]) associated with molecularly confirmed biallelic variants in the (otoferlin) *OTOF* gene, preserved outer hair cell function, and no prior cochlear implant in the same ear**.[1]

Otarmeni was approved under accelerated approval based on improvement of hearing sensitivity assessed by average pure tone audiometry at Week 24.[1] Continued approval for this indication may be contingent upon verification and description of clinical benefit in the confirmatory clinical trial.

Limitations of Use: Otarmeni is not recommended in patients in whom preoperative imaging demonstrates that access to the inner ear is not feasible, including those with abnormal mastoid pneumatization or clinically significant anatomic variations of the middle ear and inner ear.[1]

Disease Overview

In the US, congenital deafness occurs in 1.7 out of every 1,000 newborns and is mainly the result of genetic variabilities.[2] Variants in the *OTOF* gene, which encodes otoferlin, are one of the leading causes of congenital deafness; approximately 1% to 3% of congenital genetic deafness cases are due to biallelic pathogenic variants in *OTOF*.[2,3] *OTOF*-related hearing loss, an ultra-rare condition, is inherited in an autosomal recessive manner.[4] *OTOF*-related hearing loss is an auditory synaptopathy that results from defective synaptic transmission from normally functioning cochlear inner hair cells to the auditory nerve.

Otoferlin, expressed exclusively in the cochlear hair cells, vestibular hair cells, and central nervous system, is essential for auditory signal transmission.[2,3] There are no consensus clinical diagnostic criteria for *OTOF*-related hearing loss.[4] Early auditory intervention is vital to the development of speech and language. Current management options include hearing aids, cochlear implants, and assistive hearing technologies.[3,4]

Clinical Efficacy

Otarmeni is being evaluated in an ongoing, multicenter, single-arm, Phase I to II study (CHORD).[1,2] The study will enroll up to 30 patients; approval of Otarmeni was based on efficacy assessment results from 20 patients.[1] Enrolled patients were < 18 years of age and had molecularly confirmed *OTOF* gene-associated profound sensorineural hearing loss (defined by an average audiometric threshold of > 90 dB HL on pure tone audiometry averaged across 0.5, 1, 2, and 4 kHz and auditory brainstem response). In addition, patients had evidence of outer hair cell activity by otoacoustic emissions testing. Patients with cochlear implants in the same ear to be treated with Otarmeni were excluded from the study. In total, 24 patients received a single dose of Otarmeni; 10 patients received unilateral treatment and 14 patients received bilateral treatment.

The median age of the patients was 2 years (range 10 months to 16 years).[1] The majority of patients were female (n = 16) and White (n = 17), followed by Asian (n = 4) and race was not reported in 3 patients. Of

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the 24 patients who received Otarmeni, 20 patients completed efficacy assessments at Week 24; 5 patients had a contralateral cochlear implant in place before enrollment and 2 patients received a cochlear implant in the contralateral ear during the same surgical session for Otarmeni administration.

At 24 weeks, 80% of patients achieved the primary endpoint, which was the achievement of a hearing sensitivity threshold ≤ 70 dB HL assessed by average pure tone audiometry (i.e., average of pure tone audiometry thresholds at 0.5, 1, 2, and 4 kHz).[1] In total, 70% of patients achieved the key secondary endpoint, which was the presence of auditory brainstem response to a click (broadband sound) stimulus of ≤ 90 dB noise-induced hearing loss).

POLICY STATEMENT

Prior Authorization is recommended for medical benefit coverage of Otarmeni. Approval is recommended for those who meet the **Criteria** and **Dosing** for the listed indication. All approvals are provided for one dose per ear (per lifetime) [i.e., two doses per patient]. The approval duration is 90 days to allow for an adequate timeframe to administer Otarmeni. Because of the specialized skills required for evaluation and diagnosis of patients treated with Otarmeni as well as the specialized training required for administration of Otarmeni, approval requires Otarmeni to be prescribed by a physician who specializes in the condition being treated. If claims history is available, verification is required for certain criteria as noted by **[verification in claims history required]**.

All reviews (approvals and denials) will be forwarded to the Medical Director for evaluation. Some clients have elected Embarc Benefit Protection. For these clients, the Medical Director will coordinate with EviCore to ensure the Embarc Benefit Protection portion of the review has been completed. If the Embarc Benefit Protection portion of the review has not been completed, the Medical Director will route to Embarc@EviCore.com prior to completing the review.

Documentation: Documentation is required for use of Otarmeni as noted in the criteria as **[documentation required]**. Documentation may include, but is not limited to, chart notes, laboratory results, medical test results, claims records, prescription receipts, and/or other information. All documentation must include patient-specific identifying information.

Automation: None.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Otarmeni is recommended in those who meet the following criteria:

FDA-Approved Indication

Sensorineural Hearing Loss. Approve one dose per ear per lifetime (i.e., up to total of two doses) if the patient meets ALL of the following (A, B, C, D, E, F, G, and H):

- **A)** Patient is not receiving retreatment of ear(s) previously treated with Otarmeni [**verification in claims history required**]; AND
  Note: If no claims for Otarmeni is present (or if claims history is not available), the prescribing physician confirms that the patient has not previously received Otarmeni in the ear being treated.
- **B)** Patient has molecularly confirmed biallelic variants in the otoferlin (*OTOF)* gene **[documentation required]**; AND

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C) Patient has severe-to-profound or profound hearing loss, as defined by > 90 decibels hearing level **[documentation required]**; AND

D) Patient has preserved outer hair cell function, as determined by the prescriber **[documentation required]**; AND

E) According to the prescribing physician, patient did not have a prior cochlear implant in the ear to be treated with Otarmeni; AND

F) Patient meets BOTH of the following (i and ii):

i. Patient had preoperative imaging **[documentation required]**; AND

ii. Access to the inner ear is feasible **[documentation required]**; AND
Note: Scenarios such as abnormal mastoid pneumatization or clinically significant anatomic variations of the middle ear and inner ear may deem access to the inner ear infeasible.

G) The medication is prescribed by an otolaryngologist; AND

H) If criteria A though G are met, approve up to two doses of Otarmeni (one dose per ear per lifetime) to be given as an intracochlear infusion; each dose is 7.2×10^{13} vector genomes in a total volume of 0.24 mL.

Dosing. The recommended dose of Otarmeni is one dose per ear per lifetime (i.e., up to two doses per patient) to be given as an intracochlear infusion; each dose is 7.2×10^{13} vector genomes in a total volume of 0.24 mL.

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Coverage of Otarmeni is not recommended in the following situations:

1. **Prior Receipt of Gene Therapy**. Patients who had received prior gene therapy were excluded from the Otarmeni pivotal study.[1,2]

2. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

REFERENCES

1. Otarmeni™ intracochlear infusion [prescribing information]. Tarrytown NY; Regeneron: April 2026.
2. Valayannopoulos V, Bance M, Carvalho DS, et al. DB-OTO gene therapy for inherited deafness. *N Engl J Med*. 2026;394:1074-1083.
3. Yang, JH, Zhang DM, Wang K. Pathogenesis and research progress of OTOF gene related auditory neuropathy: a retrospective review. *Am J Transl Res*. 2025;17:1643-1650.
4. Azaiez H, Thorpe RK, Odell AM, et al. OTOF-related hearing loss. In: GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993. Initial posting: February 29, 2008 [updated May 21, 2026]. Available at: https://www.ncbi.nlm.nih.gov/books/NBK1251/. Accessed on September 16, 2026.

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HISTORY

Type of Revision	Summary of Changes	Review Date
New Policy	--	04/29/2026
Early Annual Revision	**Policy Statement:** Otarmeni can only be prescribed by a physician who specializes in the condition being treated; the qualifier "or in consultation with" was removed. It was clarified that approval is for up to two doses per patient. A statement about claims history was added. All reviews will be forwarded to a Medical Director for evaluation. In addition, standard language about an Embarc Benefit Protection component was added. **Documentation:** This section was added to the policy. **Sensorineural Hearing Loss:** "Verification in claims history required" and a Note regarding prescribing physician confirmation if claims history is not available were added to the requirement that patient is not receiving retreatment of ear(s) previously treated with Otarmeni. "Documentation required" was added to the following requirements: patient has molecularly confirmed biallelic variants in the otoferlin (*OTOF*) gene; patient has severe-to-profound or profound hearing loss, as defined by > 90 decibels hearing level; patient has preserved outer hair cell function, as determined by the prescriber; and patient had preoperative imaging and access to the inner ear is feasible. For the requirement that patient did not have a prior cochlear implant in the ear to be treated with Otarmeni, the qualifier "According to the prescribing physician" was added. The specialist requirement was changed to as written; previously it was The medication is prescribed by or in consultation with an otolaryngologist. A new criterion was added – that if criteria A through G are met, approve up to two doses of Otarmeni (one dose per ear per lifetime) to be given as an intracochlear infusion; each dose is 7.2×10^{13} vector genomes in a total volume of 0.24 mL. In **Conditions Not Recommended for Approval**, for "Prior Gene Therapy", the words "Receipt of" were added.	09/23/2026

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