Drug Coverage Policy

Effective Date.....08/27/2026
Coverage Policy Number.....IP0824
Policy Title.....Otarmeni

Otolaryngology – Gene Therapy – Otarmeni

- Otarmeni™ (lunsotogene parvec-cwha intracochlear infusion – Regeneron)

INSTRUCTIONS FOR USE

The following Coverage Policy applies to health benefit plans administered by Cigna Companies. Certain Cigna Companies and/or lines of business only provide utilization review services to clients and do not make coverage determinations. References to standard benefit plan language and coverage determinations do not apply to those clients. Coverage Policies are intended to provide guidance in interpreting certain standard benefit plans administered by Cigna Companies. Please note, the terms of a customer's particular benefit plan document [Group Service Agreement, Evidence of Coverage, Certificate of Coverage, Summary Plan Description (SPD) or similar plan document] may differ significantly from the standard benefit plans upon which these Coverage Policies are based. For example, a customer's benefit plan document may contain a specific exclusion related to a topic addressed in a Coverage Policy. In the event of a conflict, a customer's benefit plan document always supersedes the information in the Coverage Policies. In the absence of a controlling federal or state coverage mandate, benefits are ultimately determined by the terms of the applicable benefit plan document. Coverage determinations in each specific instance require consideration of 1) the terms of the applicable benefit plan document in effect on the date of service; 2) any applicable laws/regulations; 3) any relevant collateral source materials including Coverage Policies and; 4) the specific facts of the particular situation. Each coverage request should be reviewed on its own merits. Medical directors are expected to exercise clinical judgment where appropriate and have discretion in making individual coverage determinations. Where coverage for care or services does not depend on specific circumstances, reimbursement will only be provided if a requested service(s) is submitted in accordance with the relevant criteria outlined in the applicable Coverage Policy, including covered diagnosis and/or procedure code(s). Reimbursement is not allowed for services when billed for conditions or diagnoses that are not covered under this Coverage Policy (see "Coding Information" below). When billing, providers must use the most appropriate codes as of the effective date of the submission. Claims submitted for services that are not accompanied by covered code(s) under the applicable Coverage Policy will be denied as not covered. Coverage Policies relate exclusively to the administration of health benefit plans. Coverage Policies are not recommendations for treatment and should never be used as treatment guidelines. In certain markets, delegated vendor guidelines may be used to support medical necessity and other coverage determinations.

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.[1] 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.

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 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 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).

Coverage Policy

POLICY STATEMENT
Prior Authorization is required for benefit coverage of Otarmeni. Approval is recommended for those who meet the **Criteria** and **Dosing** for the listed indication. Extended approvals are allowed if the patient continues to meet the Criteria and Dosing. Requests for doses outside of the established dosing documented in this policy will be considered on a case-by-case basis by a clinician (i.e., Medical Director or Pharmacist). All approvals are provided for one dose per ear (per lifetime). 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 or in consultation with a physician who specializes in the condition being treated.

Otarmeni is considered medically necessary when the following criteria are met

FDA-Approved Indication

1. **Sensorineural Hearing Loss.** Approve one dose per ear (i.e., up to total of two doses) if the patient meets ALL of the following (A, B, C, D, E, F, and G):
   - **A)** Patient has molecularly confirmed biallelic variants in the *OTOF* gene; AND
   - **B)** Patient has severe-to-profound or profound hearing loss, as defined by > 90 decibels hearing level; AND
   - **C)** Patient has preserved outer hair cell function, as determined by the prescriber; AND
   - **D)** Patient did not have a prior cochlear implant in the ear to be treated with Otarmeni; AND
   - **E)** Patient is not receiving retreatment of ear(s) previously treated with Otarmeni; AND
   - **F)** Patient meets BOTH of the following (i and ii):
     - **i.** Patient had preoperative imaging; AND
     - **ii.** Access to the inner ear is feasible; 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 or in consultation with an otolaryngologist.

Dosing. Approve up to two doses of Otarmeni (one dose per ear) 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 Covered

Otarmeni for any other use is considered not medically necessary, including the following (this list may not be all inclusive; criteria will be updated as new published data are available):

1. **Retreatment of Previously Treated Ear(s)**. Otarmeni is approved for a one-time use in each ear (i.e., one intracochlear infusion per ear).[1] There are no data regarding re-treatment with Otarmeni.

Coding Information

1) This list of codes may not be all-inclusive.
2) Deleted codes and codes which are not effective at the time the service is rendered may not be eligible for reimbursement.

Considered Medically Necessary when criteria in the applicable policy statements listed above are met:

HCPCS Codes	Description
J3590	Unclassified biologics
C9399	Unclassified drugs or biologicals

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 March 13, 2025]. Available at: https://www.ncbi.nlm.nih.gov/books/NBK1251/. Accessed on April 23, 2026.

Revision Details

Summary of Changes	Review Date	Effective Date
New policy	08/27/2026	08/27/2026

The policy effective date is in force until updated or retired.