Clinical Policy: Lunsotogene Parvec-cwha (Otarmeni)

Reference Number: PA.CP.PHAR.757
Effective Date: 06/2026
Last Review Date: 05/2026

Description
Lunsotogene parvec-cwha (Otarmeni™) is a dual adeno-associated virus 1 (AAV1) vector-based gene therapy.

FDA Approved Indication(s)
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, preserved outer hair cell function, and no prior cochlear implant in the same ear.

This indication is approved under accelerated approval based on the improvement of hearing sensitivity assessed by average pure tone audiometry (PTA) at Week 24. Continued approval for this indication may be contingent upon verification and description of clinical benefit in the confirmatory clinical trial.

Limitation(s) 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.

Policy/Criteria
Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

All requests reviewed under this policy **require Precision Drug Action Committee (PDAC) Utilization Management Review**. Refer to CC.PHAR.21 for process details.

The medical necessity of Otarmeni may be determined by the clinical criteria as stated below, in addition to other factors implemented by the covering entity (Regeneron) as part of its free-of-cost drug program:

I. Initial Approval Criteria

A. Sensorineural Hearing Loss (must meet all)**:**

1. Diagnosis of severe-to-profound sensorineural hearing loss as evidenced by both of the following (a and b):
   a. Genetic testing confirms biallelic pathogenic or likely pathogenic *OTOF* variants;
   b. Member meets all of the following in the requested treatment ear(s) (i, ii, and iii):
      i. Severe-to-profound hearing loss defined by an average audiometric threshold of > 90 dB HL;
      ii. Absent auditory brainstem response (ABR);
      iii. Intact outer hair cell function as evidenced by one of the following (1 or 2, *see Appendix D*):

1. Presence of otoacoustic emissions (OAE);
2. Presence of a cochlear microphonic;

2. Prescribed by or in consultation with an otolaryngologist;
3. Member does not have a history of a cochlear implant in the requested treatment ear(s);
4. Member has not previously been treated with Otarmeni in the requested treatment ear(s);
5. Member has not received prior gene therapy;
6. Dose does not exceed a single intracochlear infusion of 7.2×10^{12} vector genomes (vg) per ear.

Approval duration: 3 months (one lifetime intracochlear infusion per ear)

B. Other diagnoses/indications

1. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

II. Continued Therapy

A. Sensorineural Hearing Loss

1. Re-authorization is not permitted. Members must meet the initial approval criteria.

Approval duration: Not applicable

B. Other diagnoses/indications (must meet 1 or 2)**:**

1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.PHARM.01) applies.
   Approval duration: Duration of request or 12 months (whichever is less); or
2. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

III. Diagnoses/Indications for which coverage is NOT authorized:

A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policies – PA.CP.PMN.53

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

ABR: auditory brainstem response
db HL: decibel hearing level
FDA: Food and Drug Administration
OAE: otoacoustic emissions
OTOF: otoferlin gene
PTA: pure tone audiometry
vg: vector genomes

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings

None reported

Appendix D: General Information

- There is currently no clinical data available on repeat administration of Otarmeni to treat an individual ear.
- Patients who did not show intact outer hair cell function were excluded from the clinical studies of Otarmeni and may not benefit from treatment based on its mechanism of action. Intact outer hair cell function can be determined by any of the following:
  - Prescence of OAE in patients generally up to 2 years of age
  - Prescence of the cochlear microphonic in patients generally older than 2 years of age

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
Severe-to-profound and profound sensorineural hearing loss associated with biallelic *OTOF*-mutations	7.2×10^{12} vg administered one time by intracochlear infusion in a total volume of 0.24 mL per ear	7.2×10^{12} vg/ear

VI. Product Availability

Single-dose vial: 3×10^{13} vg/mL

VII. References

1. Otarmeni Prescribing Information. Tarrytown, NY: Regeneron Pharmaceuticals, Inc.; April 2026. Available at: https://www.fda.gov/media/192098/download?attachment. Accessed April 29, 2026.
2. Valayannopoulos V, Bance M, Carvalho DS, et al; CHORD study group. DB-OTO gene therapy for inherited deafness. *N Engl J Med.* 2025 Oct 12. doi: 10.1056/NEJMoa2400521.
3. Bower C, Reilly BK, Richerson J, et al; Hearing assessment in infants, children, and adolescents: Recommendations beyond neonatal screening. *Pediatrics*. 2023 Sep 1;152(3):e2023063288. doi: 10.1542/peds.2023-063288.
4. Li MM, Tayoun AA, DiStefano M, et al; ACMG Professional Practice and Guidelines Committee. Clinical evaluation and etiologic diagnosis of hearing loss: A clinical practice resource of the American College of Medical Genetics and Genomics (ACMG). *Genet Med.* 2022 Jul;24(7):1392-1406. doi: 10.1016/j.gim.2022.03.018.

Coding Implications

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J3590	Unclassified biologics
C9399	Unclassified drugs or biologicals

Reviews, Revisions, and Approvals	Date
Policy created	05/2026