



Nonprofit corporations and independent licensees
of the Blue Cross and Blue Shield Association

Medical benefit drug policies are a source for BCBSM and BCN medical policy information only. These documents are not to be used to determine benefits or reimbursement. Please reference the appropriate certificate or contract for benefit information. This policy may be updated and therefore subject to change.

P&T Date: 06/11/2026

Otarmeni™ (lunsotogene parvec-cwha)

HPCPS: J3590

Policy:

Requests must be supported by submission of chart notes and patient specific documentation.

- A. Coverage of the requested drug is provided when all the following are met:
 - a. Coverage of the requested drug is considered investigational/experimental for all indications due to insufficient evidence of a clinical benefit
 - i. BCBSM and BCN are awaiting the results of ongoing clinical trials to provide evidence of a clinical benefit

***Note: Coverage and approval duration may differ for Medicare Part B members based on any applicable criteria outlined in Local Coverage Determinations (LCD) or National Coverage Determinations (NCD) as determined by Center for Medicare and Medicaid Services (CMS). See the CMS website at <http://www.cms.hhs.gov/>. Determination of coverage of Part B drugs is based on medically accepted indications which have supported citations included or approved for inclusion determined by CMS approved compendia.

Background Information:

- Otoferlin-related hearing loss is a rare, genetic, auditory neuropathy spectrum disorder (ANSD) caused by biallelic mutations in the OTOF gene. It is characterized by dysfunctional otoferlin which helps in the conduction of sound signals to the brain. Otoferlin is located within the inner hair cells of the cochlea in the inner ear. These inner hair cells act as sensory cells that convert sound vibrations into electrical signals. Otoferlin responds to an influx of calcium into inner hair cells triggering the release of neurotransmitters from vesicles within the hair cell. These neurotransmitters then carry the sound signal across the synapse to the auditory nerve. From the auditory nerve, the signal travels to the brain for interpretation as sound. Without functional otoferlin, this important step in signal transmission stops, preventing the sound information from reaching the brain even if the hair cells initially detect the sound.
- The 2016 International Pediatric Otolaryngology Group (IPOG) consensus recommendations for hearing loss in the pediatric patient recommend use of otoacoustic emissions (OAE) testing in all newborns. If newborns are at high risk for hearing loss or they have failed their initial OAE testing, an auditory brainstem response (ABR) test should then be performed. The diagnosis of ANSD is given when otoacoustic emissions and/or cochlear microphonics are present in the setting of absent or abnormal ABR. Temporal bone and brain imaging is recommended in the setting of unilateral ANSD and should be considered in the setting of bilateral ANSD. Genetic testing should be performed

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with a confirmed ANSD diagnosis as 40% - 50% of ANSD has a genetic component. Patients with otoferlin-related hearing loss may benefit from the use of cochlear implants.

- Otarmeni is an adeno-associated virus vector-based gene therapy indicated for the treatment of pediatric and adult patients with severe-to-profound and profound sensorineural hearing loss associated with molecularly confirmed biallelic variants in the OTOF gene, preserved outer hair cell function, and no prior cochlear implant in the same ear. It 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. 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.
- Safety and efficacy are being evaluated in the CHORD trial, an ongoing, multi-center, single-arm study of 24 pediatric patients with otoferlin-related hearing loss. There were two parts to the study. In part A, participants received Otarmeni in one ear and the contralateral ear could have a cochlear implant or remain untreated. In part B, participants received Otarmeni in both ears during a single session. Participants received Otarmeni at a dose of 7.2×10^{12} vector genomes in a volume of 240 μ l per dose in one ear or in each ear. Patients had to be less than 18 years of age, have biallelic OTOF gene variants, profound deafness defined by an average audiometric threshold of greater than 90 decibel hearing level [dB HL], and clinical markers of intact outer hair-cell function. Key exclusion criteria included previous gene therapy or a cochlear implant in the ear designated for Otarmeni. The primary endpoint was an average threshold on behavioral PTA at week 24 of 70 dB HL or less, a clinical standard that generally avoids cochlear implantation and enables natural acoustic hearing. A PTA average threshold of 70 dB HL or less at week 24 was found in 16 of 20 participants (80%; 95% CI: 56, 94). Auditory brain response at less than 90 dB HL at week 24 was achieved in 14 of 20 patients (70%, 95% CI: 46, 88). At week 24, a total of 9 (45%) patients achieved an average PTA threshold less than or equal to 45 dB HL, or ability to hear soft conversational speech level, and 3 (15%) patients achieved less than or equal to 25 dB HL, or normal hearing levels and the ability to hear whispers. One patient received a cochlear implant as rescue treatment approximately 8 months after Otarmeni administration in the same ear following determination of treatment failure.
- The plan considers cellular and gene therapies to be proven effective to treat a condition if the following are true: the drug has full FDA approval that was not obtained under the FDA's accelerated approval pathway and the drug met all of its primary endpoint(s) in its pivotal trials, or if confirmatory trials were required, the drug met its primary endpoint(s) in its confirmatory trial(s). Cellular and gene therapies that haven't been proven effective (do not meet the listed criteria) are considered a benefit contract exclusion for most plans.
- Otarmeni was approved under the FDA's accelerated approval pathway, and therefore, is considered experimental/investigational until further confirmatory trials are complete and the medication gets full FDA approval.

References:

1. Otarmeni [prescribing information]. Tarrytown, NY: Regeneron Pharmaceuticals, Inc.; April 2026.
2. Valayannopoulos V, Bance M, Carvalho DS, et al. DB-OTO gene therapy for inherited deafness. NEJM. 2025 Oct 12. DOI: 10.1056/NEJMoa2400521.
3. Clinicaltrials.gov. A study of DB-OTO, an adeno-associated virus (AAV) based gene therapy, in children/infants with hearing loss due to otoferlin mutations (CHORD) (NCT05788536). Available at: <https://clinicaltrials.gov/study/NCT05788536?intr=%22DB-OTO%22&viewType=Table&rank=1>. Accessed on April 26, 2026.
4. National Organization for Rare Disorders. Auditory neuropathy spectrum disorder. 2021 Aug 18. Available at: <https://rarediseases.org/rare-diseases/auditory-neuropathy-spectrum-disorder/>. Accessed on April 26, 2026.
5. Ford CL, Riggs WJ, Quigley T, et al. The natural history, clinical outcomes, and genotype–phenotype relationship of otoferlin-related hearing loss: a systematic, quantitative literature review. Hum Genet. 2023 Sept 7; 142: 1429 – 49.

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6. American Academy of Audiology. Assessment of hearing in infants and young children. 2020 Jan 23. Available at: https://www.audiology.org/wp-content/uploads/2021/05/Clin-Guid-Doc_Assess_Hear_Infants_Children_1.23.20.pdf. Accessed on April 29, 2026.
7. Alford RL, Arnos KS, Fox M, et al. American College of Medical Genetics and Genomics guideline for the clinical evaluation and etiologic diagnosis of hearing loss. Gene Med. 2024 April; 16 (4): 347 – 55.
8. Liming BJ, Carter J, Cheng A, et al. International Pediatric Otolaryngology Group (IPOG) consensus recommendations: hearing loss in the pediatric patient. Intern J Ped Otorhinolar. 2016 Nov; 90: 251 – 8.

Policy History												
#	Date	Change Description										
1.2	Effective Date: 07/01/2026	UM medical management system update for MAPPO and BCNA <table><tr><th>Line of Business</th><th>PA Required in Medical Management System (Yes/No)</th></tr><tr><td>BCBS</td><td>Yes</td></tr><tr><td>BCN</td><td>Yes</td></tr><tr><td>MAPPO</td><td>Yes</td></tr><tr><td>BCNA</td><td>Yes</td></tr></table>	Line of Business	PA Required in Medical Management System (Yes/No)	BCBS	Yes	BCN	Yes	MAPPO	Yes	BCNA	Yes
Line of Business	PA Required in Medical Management System (Yes/No)											
BCBS	Yes											
BCN	Yes											
MAPPO	Yes											
BCNA	Yes											
1.1	Effective Date: 06/11/2026	New Policy										
1.0	Effective Date: 05/04/2026	UM medical management system update for BCBS and BCN <table><tr><th>Line of Business</th><th>PA Required in Medical Management System (Yes/No)</th></tr><tr><td>BCBS</td><td>Yes</td></tr><tr><td>BCN</td><td>Yes</td></tr><tr><td>MAPPO</td><td>No</td></tr><tr><td>BCNA</td><td>No</td></tr></table>	Line of Business	PA Required in Medical Management System (Yes/No)	BCBS	Yes	BCN	Yes	MAPPO	No	BCNA	No
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BCBS	Yes											
BCN	Yes											
MAPPO	No											
BCNA	No											

* The prescribing information for a drug is subject to change. To ensure you are reading the most current information it is advised that you reference the most updated prescribing information by visiting the drug or manufacturer website or <http://dailymed.nlm.nih.gov/dailymed/index.cfm>.

Blue Cross Blue Shield/Blue Care Network of Michigan

Medication Authorization Request Form



This form is to be used by participating physicians to obtain coverage for **drugs covered under the medical benefit**. For commercial members only, please complete this form and submit via fax to 1-877-325-5979. If you have any questions regarding this process, please contact BCBSM Provider Relations and Servicing or the Medical Drug Helpdesk at 1-800-437-3803 for assistance.

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PATIENT INFORMATION	PHYSICIAN INFORMATION
Name	Name
ID Number	Specialty
D.O.B. ☐ Male ☐ Female	Address
Diagnosis	City /State/Zip
Drug Name	Phone/Fax: P: () - F: () -
Dose and Quantity	NPI
Directions	Contact Person
Date of Service(s)	Contact Person Phone / Ext.

STEP 1: DISEASE STATE INFORMATION

- Is this request for: ☐ Initiation ☐ Continuation Date patient started therapy: _____
- Administered by patient or a medical professional? ☐ patient (self) ☐ health care professional (physician, nurse, etc.)
- Site of administration? ☐ Provider office/Home infusion ☐ Other: _____
☐ Hospital outpatient facility (go to #4) Reason for Hospital Outpatient administration: _____
☐ Hospital inpatient facility for Car-T therapy only (for example: Kymriah, Yescarta, or Tecartus) (go to #5)
- Please specify location of administration if hospital outpatient infusion: _____
- Please specify location of administration if hospital inpatient infusion: _____
- Please provide the NPI number for the place of administration: _____
- Initiation AND Continuation of therapy:**
 - What is the patient's diagnosis? _____
 - What other medication has the patient received for their condition? Please list _____
 - Please describe the response to previous therapies: _____
 - Will the patient be receiving any other treatment for the listed condition while on this medication? Please list: _____
 - Please list any labs values important for diagnosing or monitoring this patient's condition: _____
- Continuation of therapy:**
 - Has the patient progressed while on this medication? ☐ yes ☐ no
 - How has the patient's condition changed while on this medication?

☐ Improved: Please describe: _____
 ☐ Stable: please describe: _____
 ☐ Worsened; Please describe: _____
 ☐ Other; Please describe: _____

Chart notes are required for the processing of all requests. Please add any other supporting medical information necessary for our review (required)

Coverage will not be provided if the prescribing physician's signature and date are not reflected on this document.

☐ Request for expedited review: I certify that applying the standard review time frame may seriously jeopardize the life or health of the member or the member's ability to regain maximum function

Physician's Name	Physician Signature	Date
Step 2: Checklist	☐ Form Completely Filled Out ☐ Provide chart notes	☐ Attach test results
Step 3: Submit	By Fax: BCBSM Specialty Pharmacy Mailbox 1-877-325-5979	By Mail: BCBSM Specialty Pharmacy Program P.O. Box 312320, Detroit, MI 48231-2320

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7/26/2018, 9/18/2018; 1/31/2020; 3/17/2020; 8/9/2021