Mepsevii® (vestronidase alfa-vjbk)
(Intravenous)

Document Number: IC-0346

Last Review Date: 04/01/2026
Date of Origin: 12/12/2017
Dates Reviewed: 12/2017, 10/2018, 02/2019, 02/2020, 02/2021, 02/2022, 02/2023, 04/2023, 02/2024, 04/2025, 04/2026

I. Length of Authorization

- Initial: Prior authorization validity will be provided initially for 12 months (365 days).
- Renewal: Prior authorization validity may be renewed every 12 months (365 days) thereafter.

II. Dosing Limits

Max Units (per dose and over time) [HCPCS Unit]:

- 460 billable units (460 mg) every 14 days

III. Initial Approval Criteria [1]

Prior authorization validity is provided in the following conditions:

- Member is at least 5 months of age; **AND**
- Documented baseline age-appropriate values for one or more of the following have been obtained: 6-minute walk test (6-MWT), motor function [i.e., Bruininks-Oseretsky Test of Motor Proficiency (BOT-2)], liver and/or spleen volume, urinary excretion of glycosaminoglycans (GAGs) such as chondroitin sulfate and dermatan sulfate, skeletal involvement (i.e. Z-score), pulmonary function tests, shoulder flexion, visual acuity; **AND**

NOTE: *For very young members in which forced vital capacity (FVC) or 6-MWT are not suitable for measuring, requests will be reviewed on a case-by-case basis.*

Universal Criteria [1,5,6]

- Therapy is being used to treat non-central nervous system manifestations of the disease and member does not have severe, irreversible cognitive impairment; **AND**

NOTE: *Requests for continued therapy in members with severe, irreversible cognitive impairment will be reviewed on a case-by-case basis.*

Mucopolysaccharidosis VII (MPS VII; Sly Syndrome) † Φ [1,2,7]

- Member has a definitive diagnosis of MPS VII as confirmed by BOTH of the following:
  - Beta-glucuronidase enzyme deficiency in peripheral blood leukocytes, fibroblasts, or dried blood spots; **AND**

- Detection of pathogenic (or likely pathogenic) variants in the *GUSB* gene by molecular genetic testing

† FDA Approved Indication(s); ‡ Compendia Recommended Indication(s); Φ Orphan Drug

IV. Renewal Criteria [1,2]

Prior authorization validity may be renewed based on the following criteria:

- Member continues to meet the universal and other indication-specific relevant criteria such as concomitant therapy requirements (not including prerequisite therapy), performance status, etc. identified in section III; **AND**
- Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include: anaphylaxis and severe allergic reactions, etc.; **AND**
- Member has demonstrated a beneficial response to therapy compared to pretreatment age-appropriate baseline values in one or more of the following:
  - Stability or improvement in 6-MWT, shoulder flexion, pulmonary function tests, visual acuity, and/or motor functions (i.e., BOT-2)
  - Reduction in liver and/or spleen volume
  - Reduction in urinary excretion of GAGs
  - Stability of skeletal disease (i.e. improvement in Z-score)

V. Dosage/Administration [1]

Indication	Dose
Mucopolysaccharidosis VII (Sly Syndrome)	4 mg/kg administered as an intravenous (IV) infusion every 2 weeks

VI. Billing Code/Availability Information

HCPCS Code:

- J3397 – Injection, vestronidase alfa-vjbk, 1 mg: 1 billable unit = 1 mg

NDC:

- Mepsevii 10 mg/5 mL single-dose vial: 69794-0001-xx

VII. References

1. Mepsevii [package insert]. Novato, CA; Ultragenyx Pharmaceutical Inc.; December 2020. Accessed March 2026.
2. Montaño AM, Lock-Hock N, Steiner RD, et al. Clinical course of sly syndrome (mucopolysaccharidosis type VII). J Med Genet. 2016 Jun;53(6):403-18.

3. Harmatz P, Whitley CB, Wang RY, et al. A novel, randomized, placebo-controlled, blind-start, single-crossover phase 3 study to assess the efficacy and safety of UX003 (rhGUS) enzyme replacement therapy in patients with MPS VII. Mol Genet Metab. 2017;120:S63.
4. Qi Y, McKeever K, Taylor J, et al. Pharmacokinetic and Pharmacodynamic Modeling to Optimize the Dose of Vestronidase Alfa, an Enzyme Replacement Therapy for Treatment of Patients with Mucopolysaccharidosis Type VII: Results from Three Trials. Clin Pharmacokinet. 2019 May;58(5):673-683. doi: 10.1007/s40262-018-0721-y.
5. Zhou J, Lin J, Leung WT, Wang L. A basic understanding of mucopolysaccharidosis: Incidence, clinical features, diagnosis, and management. Intractable Rare Dis Res. 2020 Feb;9(1):1-9. doi: 10.5582/irdr.2020.01011.
6. Shapiro EG, Eisengart JB. The natural history of neurocognition in MPS disorders: A review. Mol Genet Metab. 2021 May;133(1):8-34. doi: 10.1016/j.ymgme.2021.03.002. Epub 2021 Mar 11. PMID: 33741271.
7. Sun A, Wang R. Mucopolysaccharidosis Type VII. 2024 Jan 4. In: Adam MP, Feldman J, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2026. Available from: https://www.ncbi.nlm.nih.gov/books/NBK598990/. Accessed February 2026.

Appendix A – Non-Quantitative Treatment Limitations (NQTL) Factor Checklist

Non-quantitative treatment limitations (NQTLs) refer to the methods, guidelines, standards of evidence, or other conditions that can restrict how long or to what extent benefits are provided under a health plan. These may include things like utilization review or prior authorization. The utilization management NQTL applies comparably, and not more stringently, to mental health/substance use disorder (MH/SUD) Medical Benefit Prescription Drugs and medical/surgical (M/S) Medical Benefit Prescription Drugs. The table below lists the factors that were considered in designing and applying prior authorization to this drug/drug group, and a summary of the conclusions that Prime's assessment led to for each.

Factor	Conclusion
Indication	Yes: Consider for PA
Safety and efficacy	Yes: Consider for PA
Potential for misuse/abuse	No: PA not a priority
Cost of drug	Yes: Consider for PA

Appendix 1 – Covered Diagnosis Codes

ICD-10	ICD-10 Description
E76.29	Other mucopolysaccharidoses

Appendix 2 – Centers for Medicare and Medicaid Services (CMS)

The preceding information is intended for non-Medicare coverage determinations. Medicare coverage for outpatient (Part B) drugs is outlined in the Medicare Benefit Policy Manual (Pub. 100-2), Chapter 15, §50 Drugs and Biologicals. In addition, National Coverage Determinations (NCDs) and/or Local Coverage Determinations (LCDs) may exist and compliance with these policies is required where applicable. Local Coverage Articles (LCAs) may also exist for claims payment purposes or to clarify benefit eligibility under Part B for drugs which may be self-administered. The following link may be used to search for NCD, LCD, or LCA documents https://www.cms.gov/medicare-coverage-database/search.aspx. Additional indications, including any preceding information, may be applied at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCD/LCA): N/A

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	**Applicable State/US Territory**	**Contractor**
E (1)	CA, HI, NV, AS, GU, CNMI	Noridian Healthcare Solutions, LLC
F (2 & 3)	AK, WA, OR, ID, ND, SD, MT, WY, UT, AZ	Noridian Healthcare Solutions, LLC
5	KS, NE, IA, MO	Wisconsin Physicians Service Insurance Corp (WPS)
6	MN, WI, IL	National Government Services, Inc. (NGS)
H (4 & 7)	LA, AR, MS, TX, OK, CO, NM	Novitas Solutions, Inc.
8	MI, IN	Wisconsin Physicians Service Insurance Corp (WPS)
N (9)	FL, PR, VI	First Coast Service Options, Inc.
J (10)	TN, GA, AL	Palmetto GBA
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA
L (12)	DE, MD, PA, NJ, DC (includes Arlington & Fairfax counties and the city of Alexandria in VA)	Novitas Solutions, Inc.
K (13 & 14)	NY, CT, MA, RI, VT, ME, NH	National Government Services, Inc. (NGS)
15	KY, OH	CGS Administrators, LLC