Neighborhood Health Plan OF RHODE ISLAND™
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Mepsevii® (vestronidase alfa-vjbk)
(Intravenous)

Effective Date: 01/01/2020
Review Date: 01/22/2020, 5/27/2021, 02/17/2022, 01/19/2023, 12/07/2023, 01/04/2024, 01/15/2025
Scope: Medicaid, Commercial, Medicare-Medicaid Plan (MMP)

I. Length of Authorization

Coverage will be provided for 6 months and may be renewed for 6 months.

II. Dosing Limits

A. Quantity Limit (max daily dose) [NDC Unit]:

- Mepsevii 10 mg/5 mL vial: 46 vials per 14 days

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

- 460 billable units (460 mg) every 14 days

III. Summary of Evidence

Mepsevii is a recombinant human lysosomal beta glucuronidase indicated in pediatric and adult patients for the treatment of Mucopolysaccharidosis VII (MPS VII, Sly syndrome). Clinical trials evaluating the efficacy and safety of Mepsevii have demonstrated that patients experienced significant improvements in endurance as measured by standardized exercise tolerance tests, including the six-minute walk test (6MWT) and the three-minute stair climb test (3MSCT). Additionally, Mepsevii therapy led to reductions in liver and spleen size, improvements in skeletal manifestations such as joint stiffness and range of motion, and stabilization or improvement in respiratory function.

IV. Initial Approval Criteria [1]

Coverage is provided in the following conditions:

MMP members who have previously received this medication within the past 365 days are not subject to Step Therapy Requirements.

- Patient 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 patients in which FVC or 6-MWT are not suitable for measuring, requests will be reviewed on a case-by case basis.*

Universal Criteria [1]

- Patient does not have central nervous system manifestations of the disease and the patient does not have severe, irreversible cognitive impairment; **AND**

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

- Patient has a definitive diagnosis of MPS VII as confirmed by BOTH of the following:
  - Beta-glucuronidase enzyme deficiency in peripheral blood leukocytes; **AND**
  - Detection of pathogenic mutations in the *GUSB* gene by molecular genetic testing

† FDA-approved indication(s); **‡** Compendia recommended indication(s); **Φ** Orphan Drug

V. Renewal Criteria [1,2]

Coverage may be renewed based on the following criteria:

- Patient 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**
- Patient 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)

VI. Dosage/Administration [1]

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

VII. 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

VIII. References

1. Mepsevii [package insert]. Novato, CA; Ultragenyx Pharmaceutical Inc.; July 2022. Accessed January 2023.
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.

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)

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	**Applicable State/US Territory**	**Contractor**
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, LLC
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA, LLC
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

Policy Rationale:

Mepsevii was reviewed by the Neighborhood Health Plan of Rhode Island Pharmacy & Therapeutics (P&T) Committee. Neighborhood adopted the following clinical coverage criteria to ensure that its members use Mepsevii according to Food and Drug Administration (FDA) approved labeling and/or relevant clinical literature. Neighborhood worked with network prescribers and pharmacists to draft these criteria. These criteria will help ensure its members are using this drug for a medically accepted indication, while minimizing the risk for adverse effects and ensuring more cost-effective options are used first, if applicable and appropriate. For INTEGRITY (Medicare-Medicaid Plan) members, these coverage criteria will only apply in the absence of National Coverage Determination (NCD) or Local Coverage Determination (LCD) criteria. Neighborhood will give individual consideration to each request it reviews based on the information submitted by the prescriber and other information available to the plan.