Mepsevii® (vestronidase alfa-vjbk)
(Intravenous)

Document Number: SHP-0346

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Date of Origin: 12/12/2017
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I. Length of Authorization

Coverage will be provided for 12 months and may be renewed.

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. Initial Approval Criteria [1]

Submission of medical records (chart notes) related to the medical necessity criteria is REQUIRED on all requests for authorizations. Records will be reviewed at the time of submission. Please provide documentation related to diagnosis, step therapy, and clinical markers (i.e. genetic and mutational testing) supporting initiation when applicable. Medical records may be submitted via direct upload through the PA web portal or by fax.

Coverage is provided in the following conditions:

- 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, etc.; 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]

- Therapy is being used to treat non-central nervous system manifestations of mucopolysaccharidosis VII (MPS VII); **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

IV. 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, visual acuity, and/or other motor functions
  - Reduction in liver and/or spleen volume
  - Reduction in urinary excretion of GAGs
  - Stability of skeletal disease (i.e. improvement in Z-score)
  - Stability or improvement in pulmonary function tests, etc.

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 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.

Appendix 1 – Covered Diagnosis Codes

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

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

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 Determination (NCD), Local Coverage Determinations (LCDs), and Local Coverage Articles may exist and compliance with these policies is required where applicable. They can be found at: https://www.cms.gov/medicare-coverage-database/search.aspx. Additional indications may be covered 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, LLC
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA, LLC

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	**Applicable State/US Territory**	**Contractor**
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