Clinical Policy: Vestronidase alfa-vjbk (Mepsevii)

Reference Number: PA.CP.PHAR.374
Effective Date: 01/2018
Last Review Date: 04/2024

Description
Vestronidase alfa-vjbk (Mepsevii™) is a recombinant human lysosomal beta glucuronidase enzyme replacement therapy.

FDA Approved Indication(s)
Mepsevii is indicated in pediatric and adult patients for the treatment of Mucopolysaccharidosis VII (MPS VII, Sly syndrome).

Limitation(s) of use: The effect of Mepsevii on the central nervous system manifestations of MPS VII has not been determined.

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.

It is the policy of PA Health & Wellness that Mepsevii is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Mucopolysaccharidosis VII: Sly Syndrome (must meet all)**:**

1. Diagnosis of MPS VII (Sly syndrome) confirmed by one of the following (a or b):
   a. Two repeated enzyme assay tests demonstrating a deficiency of beta-glucuronidase;
   b. One DNA testing showing *GUSB* gene mutation;
2. Prescribed by or in consultation with a specialist with expertise in lysosomal storage diseases (e.g., pediatric endocrinologist, pediatric geneticist);
3. Apparent clinical signs of lysosomal storage disease including at least one of the following (a, b, c, or d):
   a. Enlarged liver and spleen;
   b. Joint limitations;
   c. Airway obstruction or pulmonary problems;
   d. Limitations of mobility;
4. Documentation of member's current weight (in kg);
5. Dose does not exceed 4 mg/kg IV every 2 weeks.

Approval duration: 6 months

B. Other diagnoses/indications: Refer to PA.CP.PMN.53

II. Continued Therapy

A. Mucopolysaccharidosis VII: Sly Syndrome (must meet all)**:**
1. Currently receiving medication via PA Health & Wellness benefit or member has previously met all initial approval criteria or the Continuity of Care policy (PA.LTSS.PHAR.01) applies;
2. Member is responding positively to therapy as evidenced by improvement in the individual member's MPS VII disease manifestation profile (*see Appendix D for examples*);
3. Documentation of member's current weight (in kg);
4. If request is for a dose increase, new dose does not exceed 4 mg/kg IV every 2 weeks.

Approval duration: 12 months

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.LTSS.PHAR.01) applies; or
2. Refer to PA.CP.PMN.53.

III. Appendices/General Information

Appendix A: Abbreviation/Acronym Key
FDA: Food and Drug Administration
MPS VII: mucopolysaccharidosis VII

Appendix B: Therapeutic Alternatives
Not applicable

Appendix C: Contraindications/Boxed Warnings
- Contraindication(s): none reported
- Boxed warning(s): anaphylaxis

Appendix D: General Information
- The presenting symptoms and clinical course of MPS VII can vary from one individual to another. Some examples, however, of improvement in MPS VII disease as a result of Mepsevii therapy may include improvement in:
  - 6-minute walking distance
  - Breathing difficulties
  - Muscle weakness
  - Vision or hearing problems
  - Hepatomegaly or splenomegaly
  - Reduction of total urinary glycosaminoglycan (uGAG) excretion
  - Stair climbing capacity as measured by the 3 Minute Stair Climb Test
  - Height and weight growth velocity compared to estimated pretreatment growth rate velocity from medical records for pediatric patients
- In individuals with MPS, the circulation of fluid through the blood-brain barrier may become blocked, which can lead to hydrocephalus and cortical atrophy. Seizures are a complication most common among individuals with severe forms of MPS. The clinical

benefit on this central nervous system manifestation with treatment of Mepsevii has not yet been determined.

IV. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
MPS VII (Sly syndrome)	4 mg/kg IV every 2 weeks	4 mg/kg/2 weeks

V. Product Availability

Single-dose vial: 10 mg/5 mL

VI. References

1. Mepsevii Prescribing Information. Novato, CA: Ultragenyx Pharmaceutical Inc.; December 2020. Available at: www.mepsevii.com. Accessed February 29, 2024.
2. De Oliveria Poswar F, Nehm JH, Kubaski F, et al. Diagnosis and emerging treatment strategies for mucopolysaccharidosis VII (Sly syndrome). Therapeutics and Clinical Risk Management. 2022;18:1143-55.

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
J3397	Injection, vestronidase alfa-vjbk, 10 mg

Reviews, Revisions, and Approvals	Date
Policy created	01/2018
2Q 2019 annual review: references reviewed and updated.	04/2019
2Q 2020 annual review: references reviewed and updated.	04/2020
2Q 2021 annual review: references reviewed and updated.	04/2021
2Q 2022 annual review: references reviewed and updated.	04/2022
2Q 2023 annual review: no significant changes; references reviewed and updated.	04/2023
2Q 2024 annual review: added requirement for documentation of member's weight to determine appropriate dosing for initial approval and for reauthorization; references reviewed and updated.	04/2024