## Clinical Policy: Vestronidase alfa-vjbk (Mepsevii)

Reference Number: PA.CP.PHAR.374
Effective Date: 01/2018
Last Review Date: 04/2023

Coding Implications
Revision Log

**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. 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. 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 9, 2023.

## 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 | P&T Approval Date |
|---|---|---|
| Policy created | 01/2018 | 04.18.18 |
| 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 | |