



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

Reference Number: PA.CP.PHAR.374

Effective Date: 01.09.18

Last Review Date: 04/2022

[Coding Implications](#)

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### 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 health plans affiliated with 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](http://www.mepsevii.com). Accessed February 24, 2022.

#### 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.

HCPSC Codes	Description
J3397	Injection, vestronidase alfa-vjbk, 10 mg

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created	01.09.18	04.18.18
2Q 2019 annual review: references reviewed and updated.	04.17.19	
2Q 2020 annual review: references reviewed and updated.	04/2020	
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