Clinical Policy: Vestronidase Alfa-vjbk (Mepsevii)

Reference Number: ERX.SPA.232
Effective Date: 06.01.18
Last Review Date: 05.20
Line of Business: Commercial, Medicaid

Revision Log

See Important Reminder at the end of this policy for important regulatory and legal information.

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.

Health plan approved formularies should be reviewed for all coverage determinations. Requirements to use preferred alternative agents apply only when such requirements align with the health plan approved formulary.

It is the policy of health plans affiliated with Envolve Pharmacy Solutions™ 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. 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;
3. Prescribed by or in consultation with a specialist with expertise in lysosomal storage diseases (e.g., pediatric endocrinologist, pediatric geneticist);
4. Dose does not exceed 4 mg/kg every 2 weeks.

Approval duration: 6 months

B. Other diagnoses/indications

1. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

II. Continued Therapy

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

1. Currently receiving medication via a health plan affiliated with Envolve Pharmacy Solutions or member has previously met initial approval criteria;

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 every 2 weeks.

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2)**:**

1. Currently receiving medication via a health plan affiliated with Envolve Pharmacy Solutions and documentation supports positive response to therapy.
   Approval duration: Duration of request or 6 months (whichever is less); or
2. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

III. Diagnoses/Indications for which coverage is NOT authorized:

A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off-label use policy – ERX.PA.01 or evidence of coverage documents.

IV. 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.

V. Dosage and Administration

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

VI. Product Availability

Single-dose vial: 10 mg/5 mL

VII. References

1. Mepsevii Prescribing Information. Novato, CA: Ultragenyx Pharmaceutical Inc.; December 2019. Available at: www.mepsevii.com. Accessed February 21, 2020.

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created	01.09.18	05.18
2Q 2019 annual review: no significant changes; references reviewed and updated.	02.04.19	05.19
2Q 2020 annual review: no significant changes; references reviewed and updated.	02.21.20	05.20

Important Reminder

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information.

This Clinical Policy is not intended to dictate to providers how to practice medicine, nor does it constitute a contract or guarantee regarding payment or results. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members.