Clinical Policy Title:	vestronidase alfa-vjbk
Policy Number:	RxA.217
Drug(s) Applied:	Mepsevii®
Original Policy Date:	02/07/2020
Last Review Date:	06/10/2021
Line of Business Policy Applies to:	All lines of business

Background

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

Dosing Information

Drug Name	Indication	Dosing Regimen	Maximum Dose
vestronidase alfa-vjbk (Mepsevii®)	MPS VII (Sly syndrome)	4 mg/kg IV every 2 weeks	4 mg/kg/2 weeks

Dosage Forms

- Single-dose vial: 10 mg/5 mL.

Clinical Policy

Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria. The provision of provider samples does not guarantee coverage under the terms of the pharmacy benefit administered by RxAdvance. All criteria for initial approval must be met in order to obtain coverage.

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 IV every 2 weeks.

Approval Duration
Commercial: 6 months
Medicaid: 6 months

II. Continued Therapy Approval

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

1. Member is currently receiving medication that has been authorized by RxAdvance or the member has met initial approval criteria listed in this policy ;
2. Member is responding positively to therapy (*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
Commercial: 12 months
Medicaid: 12 months

III. Appendices

APPENDIX A: Abbreviation/Acronym Key
FDA: Food and Drug Administration
MPS VII: Mucopolysaccharidosis VII
DNA: Deoxyribonucleic Acid
GUSB: Glucuronidase Beta
IV: Intravenous

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.

References

1. Mepsevii® Prescribing Information. Novato, CA: Ultragenyx Pharmaceutical Inc.; December 2020. Available at: www.mepsevii.com . Accessed March 4, 2021.

Review/Revision History	Review/Revised Date	P&T Approval Date
Policy established.	01/2020	02/07/2020
Policy was reviewed: 1. Clinical policy title table was updated. 2. Line of Business Policy Applies to was updated to all lines of business. 3. Continued therapy criteria II.A.1. was rephrased to "Currently receiving medication that has been authorized by RxAdvance…." 4. Initial and Continued therapy approval duration was updated to include Commercial and Medicaid approval duration. 5. Reference was updated.	07/05/2020	09/14/2020
Policy was reviewed: 1. Continued Therapy Approval rephrased to "Member is currently…" 2. Appendix A: Abbreviation/Acronym Key added for IV, DNA, GUSB 3. Reference were updated.	03/04/2021	06/10/2021