Prior Authorization Review Panel

CHC-MCO Policy Submission

A separate copy of this form must accompany each policy submitted for review.
Policies submitted without this form will not be considered for review.

Plan: PA Health & Wellness	**Submission Date: 05/01/2020**
Policy Number: PA.CP.PHAR.374	**Effective Date: 01/2018** **Revision Date: 04/15/2020**
Policy Name: Vestronidase alfa-vjbk (Mepsevii)	

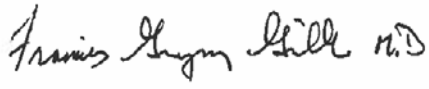
Type of Submission – Check all that apply:

- ☐ **New Policy**
- ✓ **Revised Policy***
- ☐ **Annual Review - No Revisions**
- ☐ **Statewide PDL -** *Select this box when submitting policies for Statewide PDL implementation and when submitting policies for drug classes included on the Statewide PDL.*

***All revisions to the policy must be highlighted using track changes throughout the document.**

Please provide any changes or clarifying information for the policy below:

2Q 2020 annual review: references reviewed and updated.

Name of Authorized Individual (Please type or print): **Francis G. Grillo, MD**	**Signature of Authorized Individual:** 

Clinical Policy: Vestronidase alfa-vjbk (Mepsevii)

Reference Number: PA.CP.PHAR.374
Effective Date: 01.09.18
Last Review Date: 04/2020

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 health plans affiliated with PA Health and 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. 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: 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 and 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 2019. Available at: www.mepsevii.com. Accessed February 21, 2020.

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	01.09.18	04.18.18
2Q 2019 annual review: references reviewed and updated.	04.17.19	
2Q 2020 annual review: references reviewed and updated.	04/2020	