Reference number(s)
4203-A

STANDARD MEDICARE PART B MANAGEMENT

MEPSEVII (vestronidase alfa-vjbk)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication
Mepsevii is indicated in pediatric and adult patients for the treatment of mucopolysaccharidosis VII (MPS VII, Sly syndrome).

Limitations of Use:
The effect of Mepsevii on the central nervous system manifestations of MPS VII has not been determined.

All other indications will be assessed on an individual basis. Submissions for indications other than those enumerated in this policy should be accompanied by supporting evidence from Medicare approved compendia.

II. DOCUMENTATION

The following documentation must be available, upon request, for all submissions:
A. Initial requests: beta-glucuronidase enzyme assay or genetic testing results supporting diagnosis.
B. Continuation requests: chart notes documenting a clinically positive response to therapy, which shall include improvement, stabilization, or slowing of disease progression.

III. CRITERIA FOR INITIAL APPROVAL

Mucopolysaccharidosis VII (MPS VII, Sly syndrome)
Authorization of 12 months may be granted for treatment of MPS VII (Sly syndrome) when both of the following criteria are met:
A. Diagnosis of MPS VII was confirmed by enzyme assay demonstrating a deficiency of beta-glucuronidase enzyme activity or by genetic testing; AND
B. Elevated urinary glycosaminoglycan (uGAG) excretion at a minimum of 2-fold over the mean normal for age at initiation of treatment with Mepsevii.

IV. CONTINUATION OF THERAPY

All members (including new members) requesting authorization for continuation of therapy must be currently receiving therapy with the requested agent.

Authorization of 12 months may be granted when all of the following criteria are met:

A. The member is currently receiving therapy with Mepsevii
B. Mepsevii is being used to treat an indication enumerated in Section III
C. The member is receiving benefit from therapy. Benefit is defined as a clinically positive response to therapy, which shall include improvement, stabilization, or slowing of disease progression.

V. SUMMARY OF EVIDENCE

The contents of this policy were created after examining the following resources:

1. The prescribing information for Mepsevii.
2. The available compendium
    a. National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium
    b. Micromedex DrugDex
    c. American Hospital Formulary Service- Drug Information (AHFS-DI)
    d. Lexi-Drugs
    e. Clinical Pharmacology

After reviewing the information in the above resources, the FDA-approved indications listed in the prescribing information for Mepsevii are covered.

VI. EXPLANATION OF RATIONALE

Support for FDA-approved indications can be found in the manufacturer's prescribing information.

Support for using enzyme assay or genetic testing prior to initiating Mepsevii to treat MPS VII can be found in the trials cited in the prescribing information. To be included in the trial, the patient must have either had the diagnosis of MPS VII based on leukocyte or fibroblast glucuronidase enzyme assay or genetic testing. Additionally, treatment-naïve patients had to have an elevated urinary glycosaminoglycans (uGAG) excretion at a minimum of 2-fold over normal.

VII. REFERENCES

1. Mepsevii [package insert]. Novato, CA: Ultragenyx; December 2020.
2. ClinicalTrials.gov [Internet]. Bethesda (MD): National Library of Medicine (US). Identifier NCT01856218. An OpenLabel Phase 1/2 Study to Assess the Safety, Efficacy and Dose of Study Drug UX003 Recombinant Human Beta- glucuronidase (rhGUS) Enzyme Replacement Therapy in Patients With Mucopolysaccharidosis Type 7 (MPS 7); January 31, 2018. Available at: https://clinicaltrials.gov/ct2/show/NCT01856218?term=NCT01856218&rank=1.
3. ClinicalTrials.gov [Internet]. Bethesda (MD): National Library of Medicine (US). Identifier NCT02230566. A Phase 3 Study of UX003 Recombinant Human Betaglucuronidase (rhGUS) Enzyme Replacement Therapy in Patients With Mucopolysaccharidosis Type 7 (MPS 7); February 16, 2018. Available at: https://clinicaltrials.gov/ct2/show/NCT02230566?term=NCT02230566&rank=1.
4. ClinicalTrials.gov [Internet]. Bethesda (MD): National Library of Medicine (US). Identifier NCT02432144. A LongTerm Open-Label Treatment and Extension Study of UX003 rhGUS Enzyme Replacement Therapy in Subjects With MPS 7; November 6, 2017. Available at: https://clinicaltrials.gov/ct2/show/NCT02432144?term=NCT02432144&rank=1.
5. Harmatz P, et al. A novel Blind Start study design to investigate vestronidase alfa for mucopolysaccharidosis VII, an ultra-rare genetic disease. Mol Genet Metab. 2018 Apr;123(4):488-494.