POLICY Document for Mepsevii

The overall objective of this policy is to support the appropriate and cost effective use of the medication, lower cost site of care and overall clinically appropriate use. This document provides specific information to each section of the overall policy.

Section 1: Site of Care
- Policy information specific to site of care (outpatient, hospital outpatient, home infusion)

Section 2: Clinical Criteria
- Policy information specific to the clinical appropriateness for the medication

Section 1: Site of Care

Site of Care Criteria
Administration of Intravenous Mepsevii

POLICY

I. CRITERIA FOR APPROVAL FOR ADMINISTRATION IN OUTPATIENT HOSPITAL SETTING

This policy provides coverage for administration of Mepsevii in an outpatient hospital setting for up to 50 days when a member is new to therapy or is reinitiating therapy after not being on therapy for at least 6 months.

This policy provides coverage for administration of Mepsevii in an outpatient hospital setting for a longer course of treatment when ANY of the following criteria are met:

A. The member has experienced an adverse reaction to the drug that did not respond to conventional interventions (eg, acetaminophen, steroids, diphenhydramine, fluids or other pre-medications) or a severe adverse event (anaphylaxis, anaphylactoid reactions, myocardial infarction, thromboembolism, or seizures) during or immediately (including up to 60 minutes) after an infusion.
B. The member is medically unstable (eg respiratory, cardiovascular, or renal conditions).
C. The member has severe venous access issues that require the use of special interventions only available in the outpatient hospital setting.
D. The member has significant behavioral issues and/or physical or cognitive impairment that would impact the safety of the infusion therapy AND the patient does not have access to a caregiver.
E. The member is less than 21 years of age or is 65 years of age or older..

For situations where administration of Mepsevii does not meet the criteria for outpatient hospital infusion, coverage for Mepsevii is provided when administered in alternative sites such as; physician office, home infusion or ambulatory care.

II. REQUIRED DOCUMENTATION

The following information is necessary to initiate the site of care prior authorization review (where applicable):

A. Medical records supporting the member has experienced an adverse reaction that did not respond to conventional interventions or a severe adverse event during or immediately after an infusion
B. Medical records supporting the member is medically unstable
C. Medical records supporting the member has severe venous access issues that requires specialized interventions only available in the outpatient hospital setting
D. Medical records supporting the member has behavioral issues and/or physical or cognitive impairment and no access to a caregiver

Section 2: Clinical Criteria

SPECIALTY GUIDELINE 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).

All other indications are considered experimental/investigational and not medically necessary.

II. REQUIRED DOCUMENTATION
Submission of the following information is necessary to initiate the prior authorization review: beta-glucuronidase enzyme assay or genetic testing results supporting diagnosis.

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

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for mucopolysaccharidosis VII (MPS VII, Sly syndrome) who are responding to therapy (e.g., improvement, stabilization, or slowing of disease progression for motor function, pulmonary function, reduction in liver volume, reduction in spleen volume).

REFERENCES:

SECTION 1

1. Mepsevii [package insert]. Novato, CA: Ultragenyx Pharmaceutical Inc.; November 2017.
2. A Phase 3 Study of UX003 Recombinant Human Betaglucuronidase (rhGUS) Enzyme Replacement Therapy in Patients With Mucopolysaccharidosis Type 7 (MPS 7). https://ClinicalTrials.gov/show/NCT02230566.

SECTION 2

3. Mepsevii [package insert]. Novato, CA: Ultragenyx; November 2017.
4. 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.
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6. 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.
7. 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.