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POLICY Document for MEPSEVII (vestronidase alfa-vjbk)

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

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated.

Brand Name	Generic Name	Dosage Form
Mepsevii	vestronidase alfa-vjbk	intravenous

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:

- The member has experienced an adverse reaction to the drug that did not respond to conventional interventions (e.g., 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.

- The member is medically unstable (e.g., respiratory, cardiovascular, or renal conditions).
- The member has severe venous access issues that require the use of special interventions only available in the outpatient hospital setting.
- 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.
- Alternative infusion sites (pharmacy, physician office, ambulatory care, etc.) are greater than 30 miles from the member's home.
- The member is less than 14 years of age.

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.

Required Documentation

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

- 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
- Medical records supporting the member is medically unstable
- Medical records supporting the member has severe venous access issues that requires specialized interventions only available in the outpatient hospital setting
- Medical records supporting the member has behavioral issues and/or physical or cognitive impairment and no access to a caregiver
- Records supporting alternative infusion sites are greater than 30 miles from the member's home

Medical records supporting the member is new to therapy

Section 2: Clinical Criteria

Specialty Guideline Management
Mepsevii

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Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over the counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Mepsevii	vestronidase alfa-vjbk

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 Indications[1]

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 are considered experimental/investigational and not medically necessary.

Documentation

Submission of the following information is necessary to initiate the prior authorization review:

- Initial requests: beta-glucuronidase enzyme assay or genetic testing results supporting diagnosis.
- Continuation requests: chart notes documenting a clinically positive response to therapy, which shall include improvement, stabilization, or slowing of disease progression.

Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of metabolic disease and/or lysosomal storage disorders.

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Coverage Criteria

Mucopolysaccharidosis VII (MPS VII, Sly syndrome)[1-5]

Authorization of 12 months may be granted for treatment of MPS VII (Sly syndrome) when both of the following criteria are met:

- Diagnosis of MPS VII was confirmed by enzyme assay demonstrating a deficiency of beta-glucuronidase enzyme activity or by genetic testing; AND
- Member has elevated urinary glycosaminoglycan (uGAG) excretion at a minimum of 2-fold over the mean normal for age at initiation of treatment with the requested medication.

Continuation of Therapy

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in the Coverage Criteria section who have a clinically positive response to therapy, which shall include improvement, stabilization, or slowing of disease progression.

REFERENCES

SECTION 1

1. Mepsevii [package insert]. Novato, CA: Ultragenyx Pharmaceutical Inc.; December 2020.
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 Pharmaceutical Inc.; December 2020.
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.
5. 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.

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.