Reference number(s)
2415-A

Specialty Guideline Management
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. 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.
- Chart notes or medical records documenting clinical signs and symptoms of disease at baseline (e.g., cholestatic jaundice, hepatosplenomegaly, characteristic musculoskeletal features, developmental delay/intellectual disability, characteristic craniofacial features, recurrent otitis media or respiratory infections, hearing loss, hernias, characteristic cardiovascular abnormalities).

Continuation Requests:

- Chart notes or medical records documenting a clinically positive response to therapy, which shall include improvement, stabilization, or slowing of disease progression (e.g., improvement, stabilization, or slowing of disease progression in urinary glycosaminoglycan (uGAG) excretion, 6-minute walk test, predicted forced vital capacity, maximum ventilatory ventilation, shoulder flexion and extension maximum range, visual acuity).

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.

Coverage Criteria

Mucopolysaccharidosis VII (MPS VII, Sly syndrome)[1-6]

Authorization of 12 months may be granted for treatment of MPS VII (Sly syndrome) when all 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.
- 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.
- Member exhibits clinical signs and symptoms of disease at baseline (e.g., cholestatic jaundice, hepatosplenomegaly, characteristic musculoskeletal features, developmental delay/intellectual disability, characteristic craniofacial features, recurrent otitis media or respiratory infections, hearing loss, hernias, characteristic cardiovascular abnormalities).

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

(e.g., improvement, stabilization, or slowing of disease progression in urinary glycosaminoglycan (uGAG) excretion, 6-minute walk test, predicted forced vital capacity, maximum ventilatory ventilation, shoulder flexion and extension maximum range, visual acuity).

References

1. Mepsevii [package insert]. Novato, CA: Ultragenyx Pharmaceutical Inc.; December 2020.
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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.
6. Sun A, Wang R. Mucopolysaccharidosis Type VII. 2024 Jan 4. In: Adam MP, Bick S, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2026. Available from: https://www.ncbi.nlm.nih.gov/books/NBK598990/