PHARMACY POLICY STATEMENT
Indiana Medicaid

DRUG NAME	Mepsevii (vestronidase alfa-vjbk)
BENEFIT TYPE	Medical
STATUS	Prior Authorization Required

Mepsevii is an enzyme replacement therapy (ERT) that was approved by the FDA in 2017 for the treatment of Sly Syndrome, also known as mucopolysaccharidosis type VII (MPS VII).
MPS VII is a very rare, lysosomal storage disorder. Mutations of the GUSB gene cause deficiency of the enzyme beta glucuronidase. Normally this lysosomal enzyme breaks down glycosaminoglycans (GAGs) (previously known as mucopolysaccharides) but when reduced in MPS VII, the GAG substrates heparan sulfate (HS), dermatan sulfate (DS), and chondroitin sulfate (CS) accumulate throughout the body causing cellular and organ dysfunction. A distinguishing clinical feature of this type of MPS is the common presence of hydrops fetalis (excess accumulation of fluids in the body) in severe phenotypes.

Mepsevii (vestronidase alfa-vjbk) will be considered for coverage when the following criteria are met:

Mucopolysaccharidosis VII (MPS VII; Sly Syndrome)

For **initial** authorization:
1. Medication must be prescribed by or in consultation with a geneticist, metabolic specialist, or pediatrician experienced with managing mucopolysaccharidoses; AND
2. Member has a diagnosis of MPS VII confirmed by at least one of the following:
   a) Low beta-glucuronidase (GUS) enzyme activity
   b) Molecular genetic testing reveals pathogenic mutation of the GUSB gene; AND
3. Member has elevated urinary glycosaminoglycan (uGAG) excretion.
4. **Dosage allowed/Quantity limit:** 4 mg/kg administered by intravenous infusion every two weeks

If all the above requirements are met, the medication will be approved for 6 months.

For **reauthorization**:
1. Chart notes must show reduced uGAG excretion level; AND
2. Improvement or stabilization of at least one of the following compared to baseline: six-minute walk test (6MWT), forced vital capacity (FVC), motor function, visual acuity, hepatosplenomegaly, or fatigue.

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Mepsevii (vestronidase alfa-vjbk) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
09/13/2018	New policy for Mepsevii created.
07/27/2021	Transferred to new template. Updated J code. Added home and office to sites of service. Updated references. Added specialist requirement. Clarified diagnosis requirement. Removed baseline multi domain testing. Changed initial approval duration from 12 months to 6 months. Edited renewal criteria to reflect efficacy results from clinical trials. Removed bone marrow/stem cell transplant exclusion.
12/15/2023	Updated references. Removed specific level of uGAG elevation required.
05/13/2026	Annual review; no updates.

References:

1. Mepsevii [package insert]. Novato, CA: Ultragenyx Pharmaceutical Inc.; Revised 12/2020.
2. 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.
3. Wang RY, da Silva Franco JF, López-Valdez J, et al. The long-term safety and efficacy of vestronidase alfa, rhGUS enzyme replacement therapy, in subjects with mucopolysaccharidosis VII [published correction appears in Mol Genet Metab. 2020 Sep - Oct;131(1-2):285]. *Mol Genet Metab*. 2020;129(3):219-227. doi:10.1016/j.ymgme.2020.01.003
4. Lehman TJ, Miller N, Norquist B, Underhill L, Keutzer J. Diagnosis of the mucopolysaccharidoses. *Rheumatology (Oxford)*. 2011;50 Suppl 5:v41-v48. doi:10.1093/rheumatology/ker390
5. McCafferty EH, Scott LJ. Vestronidase Alfa: A Review in Mucopolysaccharidosis VII [published correction appears in BioDrugs. 2019 Apr 16;:]. *BioDrugs*. 2019;33(2):233-240. doi:10.1007/s40259-019-00344-7
6. Lau HA, Viskochil D, Tanpaiboon P, et al. Long-term efficacy and safety of vestronidase alfa enzyme replacement therapy in pediatric subjects < 5 years with mucopolysaccharidosis VII. *Mol Genet Metab*. 2022;136(1):28-37. doi:10.1016/j.ymgme.2022.03.002
7. Kubaski F, de Oliveira Poswar F, Michelin-Tirelli K, et al. Diagnosis of Mucopolysaccharidoses. *Diagnostics (Basel).* 2020;10(3):172. Published 2020 Mar 22. doi:10.3390/diagnostics10030172
8. Poswar FO, Henriques Nehm J, Kubaski F, Poletto E, Giugliani R. Diagnosis and Emerging Treatment Strategies for Mucopolysaccharidosis VII (Sly Syndrome). *Ther Clin Risk Manag*. 2022;18:1143-1155. Published 2022 Dec 22. doi:10.2147/TCRM.S351300

Effective date: 11/01/2026
Revised date: 05/13/2026