

PHARMACY POLICY STATEMENT

Ohio Medicaid

DRUG NAME

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.

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
9. Ohio Administrative Code. (2022, February 23). 5160-1-01 (C) Medicaid medical necessity: definitions and principles. Retrieved February 22 2023 from codes.ohio.gov.
10. Ohio Administrative Code. (2022, July 18). 5160-26-03 Managed care: covered services. Retrieved February 22, 2023 from codes.ohio.gov.
11. Ohio Administrative Code. (2020, January 1). 5160-9-03 Pharmacy services: covered drugs and associated limitations. Retrieved February 22, 2023 from codes.ohio.gov.

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