HAP CareSource™

PHARMACY POLICY STATEMENT
HAP CareSource™ Marketplace

DRUG NAME	Vimizim (elosulfase alfa)
BENEFIT TYPE	Medical
STATUS	Prior Authorization Required

Vimizim is an enzyme replacement therapy that was approved by the FDA in 2014 for the treatment of Mucopolysaccharidosis type IVA, also known as MPS IVA or Morquio A syndrome.
MPS IVA is a rare, genetic lysosomal storage disease. Pathogenic mutations of the GALNS gene cause the enzyme N-acetylgalactosamine-6-sulfatase (GALNS) to be deficient or absent. Normally this lysosomal enzyme breaks down glycosaminoglycans (GAGs) (previously known as mucopolysaccharides) but when it is reduced in MPS IVA, the GAG substrates keratan sulfate (KS) and chondroitin-6-sulfate (C6S) accumulate throughout the body to cause cellular, tissue, and organ dysfunction, notably skeletal deformities. Vimizim provides an exogenous source of GALNS.

Vimizim (elosulfase alfa) will be considered for coverage when the following criteria are met:

Mucopolysaccharidosis type IVA (MPS IVA; Morquio A 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 IVA confirmed by at least one of the following:
   a) Low GALNS enzyme activity (in fibroblasts or leukocytes) AND normal activity of a second sulfatase (to exclude Multiple Sulfatase Deficiency), and/or
   b) Molecular genetic testing identifies pathogenic GALNS gene mutations; AND
3. Documentation of baseline urinary KS (uKS) level.
4. **Dosage allowed/Quantity limit:** 2 mg/kg IV infusion once weekly

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

For **reauthorization**:
1. Chart notes must show improvement or stabilized signs and symptoms of disease such as improved endurance (e.g., 6-minute walk test) and/or reduced uKS levels.

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

HAP CareSource considers Vimizim (elosulfase alfa) 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
07/20/2021	New policy for Vimizim created.
12/28/2023	Updated references. Specified fibroblasts or leukocytes for enzyme activity measure.

References:

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4. Wood TC, Harvey K, Beck M, et al. Diagnosing mucopolysaccharidosis IVA. *J Inherit Metab Dis*. 2013;36(2):293-307. doi:10.1007/s10545-013-9587-1
5. Jones SA, Bialer M, Parini R, et al. Safety and clinical activity of elosulfase alfa in pediatric patients with Morquio A syndrome (mucopolysaccharidosis IVA) less than 5 y. *Pediatr Res*. 2015;78(6):717-722. doi:10.1038/pr.2015.169
6. Hendriksz CJ, Burton B, Fleming TR, et al. Efficacy and safety of enzyme replacement therapy with BMN 110 (elosulfase alfa) for Morquio A syndrome (mucopolysaccharidosis IVA): a phase 3 randomised placebo-controlled study. *J Inherit Metab Dis*. 2014;37(6):979-990. doi:10.1007/s10545-014-9715-6
7. Mitchell JJ, Burton BK, Bober MB, et al. Findings from the Morquio A Registry Study (MARS) after 6 years: Long-term outcomes of MPS IVA patients treated with elosulfase alfa. *Mol Genet Metab*. 2022;137(1-2):164-172. doi:10.1016/j.ymgme.2022.08.007
8. Magner M, Almássy Z, Gucev Z, et al. Consensus statement on enzyme replacement therapy for mucopolysaccharidosis IVA in Central and South-Eastern European countries. *Orphanet J Rare Dis*. 2022;17(1):190. Published 2022 May 10. doi:10.1186/s13023-022-02332-7
9. Hendriksz CJ, Harmatz P, Beck M, et al. Review of clinical presentation and diagnosis of mucopolysaccharidosis IVA. *Mol Genet Metab*. 2013;110(1-2):54-64. doi:10.1016/j.ymgme.2013.04.002

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