HAP CareSource™

PHARMACY POLICY STATEMENT
HAP CareSource™ Marketplace

DRUG NAME	Naglazyme (galsulfase)
BENEFIT TYPE	Medical
STATUS	Prior Authorization Required

Naglazyme is an enzyme replacement therapy that was approved by the FDA in 2005 for treating Mucopolysaccharidosis VI, also known as MPS VI or Maroteaux-Lamy syndrome.
MPS VI is a rare, genetic lysosomal storage disease. Pathogenic mutations of the ARSB gene cause the enzyme arylsulfatase B (ASB) (also known as N-acetylgalactosamine-4-sulfatase) to be deficient or absent. Usually this lysosomal enzyme breaks down glycosaminoglycans (GAGs) (previously known as mucopolysaccharides) but when it is reduced in MPS VI, the GAG substrate dermatan sulfate accumulates throughout the body to cause progressive multi-systemic damage and dysfunction. Clinically, Naglazyme has been shown to improve walking and stair-climbing capacity.

Naglazyme (galsulfase) will be considered for coverage when the following criteria are met:

Mucopolysaccharidosis VI (MPS VI; Maroteaux-Lamy 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 VI confirmed by at least one of the following:
   a) ASB enzyme activity is less than 10% of the lower limit of normal AND activity of a second sulfatase is normal (to exclude Multiple Sulfatase Deficiency), and/or
   b) Molecular genetic analysis identifying mutations of the ARSB gene; AND
3. Chart notes must include baseline urinary GAG (uGAG) levels showing elevated dermatan sulfate.
4. **Dosage allowed/Quantity limit:** 1 mg/kg once weekly as an IV infusion

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. Chart notes must show improvement or stabilized signs and symptoms of disease such as improved endurance (e.g., walk test or stair climb) or improved pulmonary function (e.g., FVC or FEV1).

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

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HAP CareSource considers Naglazyme (galsulfase) 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
07/09/2021	New policy for Naglazyme created.
12/27/2023	Updated references. Changed renewal to require both uGAG reduction and clinical response. Added pulmonary response as an option for renewal.

References:

1. Naglazyme [package insert]. Novato, CA: BioMarin Pharmaceutical Inc.; 2019.
2. Wood T, Bodamer OA, Burin MG, et al. Expert recommendations for the laboratory diagnosis of MPS VI. *Mol Genet Metab*. 2012;106(1):73-82. doi:10.1016/j.ymgme.2012.02.005
3. Akyol MU, Alden TD, Amartino H, et al. Recommendations for the management of MPS VI: systematic evidence- and consensus-based guidance. *Orphanet J Rare Dis*. 2019;14(1):118. Published 2019 May 29. doi:10.1186/s13023-019-1080-y
4. Brunelli MJ, Atallah ÁN, da Silva EM. Enzyme replacement therapy with galsulfase for mucopolysaccharidosis type VI. *Cochrane Database Syst Rev*. 2021;9(9):CD009806. Published 2021 Sep 17. doi:10.1002/14651858.CD009806.pub3
5. Harmatz PR, Lampe C, Parini R, et al. Enzyme replacement therapy outcomes across the disease spectrum: Findings from the mucopolysaccharidosis VI Clinical Surveillance Program. *J Inherit Metab Dis*. 2019;42(3):519-526. doi:10.1002/jimd.12079
6. Harmatz P, Shediac R. Mucopolysaccharidosis VI: pathophysiology, diagnosis and treatment. *Front Biosci (Landmark Ed)*. 2017;22:385-406. Published 2017 Jan 1. doi:10.2741/4490
7. D'Avanzo F, Zanetti A, De Filippis C, Tomanin R. Mucopolysaccharidosis Type VI, an Updated Overview of the Disease. *Int J Mol Sci*. 2021;22(24):13456. Published 2021 Dec 15. doi:10.3390/ijms222413456

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