

Specialty Guideline Management

Naglazyme

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
Naglazyme	galsulfase

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 Indication¹

Naglazyme is indicated for patients with Mucopolysaccharidosis VI (MPS VI, Maroteaux-Lamy syndrome). Naglazyme has been shown to improve walking and stair-climbing capacity.

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:

- N-acetylgalactosamine-4-sulfatase (arylsulfatase B) enzyme assay or genetic testing results supporting diagnosis.
- Chart notes or medical records documenting clinical signs and symptoms of disease at baseline (e.g., decreased growth velocity, coarse facial features, skeletal deformities, frequent upper-airway infections, enlarged liver and spleen, hearing loss, joint stiffness, coarse hair).

Continuation Requests:

- Chart notes 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 for growth rate, 12-minute walk test, 3-minute stair climb test, urinary glycosaminoglycan (GAG) levels).

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 VI (MPS VI, Maroteaux-Lamy syndrome)¹⁻³

Authorization of 12 months may be granted for treatment of MPS VI (Maroteaux-Lamy syndrome) when both of the following criteria are met:

- The diagnosis of MPS VI was confirmed by enzyme assay demonstrating a deficiency of N-acetylgalactosamine-4-sulfatase (arylsulfatase B) enzyme activity or by genetic testing.
- Member exhibits clinical signs and symptoms of disease at baseline (e.g., decreased growth velocity, coarse facial features, skeletal deformities, frequent upper-airway infections, enlarged liver and spleen, hearing loss, joint stiffness, coarse hair).

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 for growth rate, 12-minute walk test, 3-minute stair climb test, urinary glycosaminoglycan (GAG) levels).

References

1. Naglazyme [package insert]. Novato, CA: BioMarin Pharmaceutical Inc.; September 2024.
2. Akyol, M.U., Alden, T.D., Amartino, H. et al. Recommendations for the management of MPS VI: systematic evidence- and consensus-based guidance. *Orphanet J Rare Dis* 14, 118 (2019).
3. Decker C, Yu ZF, Giugliani R, et al. Enzyme replacement therapy for mucopolysaccharidosis VI: Growth and pubertal development in patients treated with recombinant human N-acetylgalactosamine 4-sulfatase. *J Pediatr Rehabil Med*. 2010;3(2):89-100. doi:10.3233/PRM-2010-0113