POLICY Document for NAGLAZYME (galsulfase)

The overall objective of this policy is to support the appropriate and cost-effective use of the medication, specific to use of preferred medication options, lower cost site of care and overall, clinically appropriate use. This document provides specific information to each of the three sections of the overall policy.

Section 1: Site of Care

- Policy information specific to site of care (outpatient, hospital outpatient, home infusion)

Section 2: Clinical Criteria

- Policy information specific to the clinical appropriateness for the medication

Section 1: Site of Care

Site of Care Criteria
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.

Brand Name	Generic Name	Dosage Form
Naglazyme	galsulfase	intravenous

Criteria For Approval For Administration In Outpatient Hospital Setting

This policy provides coverage for administration of Naglazyme in an outpatient hospital setting for up 54 days when a member is new to therapy or is reinitiating therapy after not being on therapy for at least 6 months.

This policy provides coverage for administration of Naglazyme in an outpatient hospital setting for a longer course of treatment when ANY of the following criteria are met:

- The member has experienced an adverse reaction to the drug that did not respond to conventional interventions (e.g., acetaminophen, steroids, diphenhydramine, fluids, other

pre-medications or slowing of infusion rate) or a severe adverse event (anaphylaxis, anaphylactoid reactions, myocardial infarction, thromboembolism, or seizures) during or immediately after an infusion (up to 24 hours post infusion).
- The member is medically unstable (e.g., respiratory, cardiovascular, or renal conditions).
- The member has severe venous access issues that require the use of special interventions only available in the outpatient hospital setting.
- The member has significant behavioral issues and/or physical or cognitive impairment that would impact the safety of the infusion therapy AND the patient does not have access to a caregiver.
- Alternative infusion sites (pharmacy, physician office, ambulatory care, etc.) are greater than 30 miles from the member's home.
- The member is less than 14 years of age.

For situations where administration of Naglazyme does not meet the criteria for outpatient hospital infusion, coverage for Naglazyme is provided when administered in alternative sites such as; physician office, home infusion or ambulatory care.

Documentation

The following information is necessary to initiate the site of care prior authorization review (where applicable):

- Medical records supporting the member has experienced an adverse reaction that did not respond to conventional interventions or a severe adverse event during or immediately after an infusion
- Medical records supporting the member is medically unstable
- Medical records supporting the member has severe venous access issues that requires specialized interventions only available in the outpatient hospital setting
- Medical records supporting the member has behavioral issues and/or physical or cognitive impairment and no access to a caregiver
- Records supporting alternative infusion sites are greater than 30 miles from the member's home
- Medical records supporting the member is new to therapy

Section 2: Clinical Criteria

1. Specialty Guideline Management Naglazyme

CVS/caremark™

2.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

3.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.

4. FDA-Approved Indication[1]

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.

5.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.
- Continuation requests: chart notes documenting a clinically positive response to therapy, which shall include improvement, stabilization, or slowing of disease progression.

6.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.

7.Coverage Criteria

8. Mucopolysaccharidosis VI (MPS VI, Maroteaux-Lamy syndrome)[1,2]

Authorization of 12 months may be granted for treatment of MPS VI (Maroteaux-Lamy syndrome) when 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.

9.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.

REFERENCES

SECTION 1

1. Naglazyme [package insert]. Novato, CA: BioMarin Pharmaceutical Inc; July 2024.
2. Bagewadi S, Roberts J, Mercer J, Jones S, Stephenson J, Wraith JE. Home treatment with Elaprase and Naglazyme is safe in patients with mucopolysaccharidoses types II and VI, respectively. *J Inherit Metab Dis*. 2008;31(6):733-737.
3. Harmatz P, Ketteridge D, Giugliani R, et al. Direct comparison of measures of endurance, mobility, and joint function during enzyme-replacement therapy of mucopolysaccharidosis VI (Maroteaux-Lamy syndrome): results after 48 weeks in a phase 2 open-label clinical study of recombinant human N-acetylgalactosamine 4-sulfatase. *Pediatrics*. 2005;115(6):e681-689.

SECTION 2

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).