Medical Drug Clinical Criteria

Subject:	Naglazyme (galsulfase)		
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Overview

This document addresses Naglazyme (galsulfase), an enzyme replacement therapy approved by the Food and Drug Administration (FDA) to treat individuals with Mucopolysaccharidosis VI (Maroteaux-Lamy syndrome).

The mucopolysaccharidoses are a group of inherited metabolic diseases caused by the deficiency of lysosomal enzymes needed to breakdown mucopolysaccharides or glycosaminoglycan (GAGs). The progressive accumulation of GAGs in lysosomes leads to respiratory, cardiac, skeletal and connectivity, neurologic and ophthalmologic complications. There are seven distinct types of mucopolysaccharidosis (I, II, III, IV, VI, VII, and IX). Accurate diagnosis is important to provide disease-specific enzyme replacement therapy. Diagnosis is confirmed through urinary GAG concentration measurement, enzymatic activity measurement or genetic testing.

Naglazyme has a black box warning for hypersensitivity reactions. Anaphylaxis has occurred during the early course of enzyme replacement therapy and after extended duration of therapy. Appropriate medical support should be available during Naglazyme administration. Individuals should be educated on the signs and symptoms of anaphylaxis and to seek immediate medical care should they occur.

Clinical Criteria

When a drug is being reviewed for coverage under a member's medical benefit plan or is otherwise subject to clinical review (including prior authorization), the following criteria will be used to determine whether the drug meets any applicable medical necessity requirements for the intended/prescribed purpose.

Naglazyme (galsulfase)

Initial requests for Naglazyme (galsulfase) may be approved if the following criteria are met:

I. Individual has a diagnosis of Mucopolysaccharidosis VI (Maroteaux-Lamy syndrome) demonstrated by (Akyol 2019, Wood 2012):
   A. Documentation is provided showing an increase in dermatan sulfate in the urine and a decrease in the activity of N-acetylgalactosamine-4-sulfatase (arylsulfatase B) enzyme as measured in fibroblasts or leukocytes *combined with* normal enzyme activity level of another sulfatase; **OR**
   B. Documentation is provided showing an N-acetylgalactosamine-4-sulfatase (arylsulfatase B) gene mutation.

Continuation requests for Naglazyme (galsulfase) may be approved if the following criterion is met:

I. Documentation is provided that there is clinically significant improvement or stabilization in clinical signs and symptoms of disease (including but not limited to reduction in urinary GAG excretion, reduction in hepatosplenomegaly, improvement in pulmonary function, improvement in walking distance and/or improvement in fine or gross motor function) compared to the predicted natural history trajectory of disease.

Naglazyme (galsulfase) may not be approved when the above criteria are not met and for all other indications.

Quantity Limits

Naglazyme (galsulfase) Quantity Limit

Drug	Limit
Naglazyme (galsulfase) 5 mg vial	1 mg/kg once a week

Coding

The following codes for treatments and procedures applicable to this document are included below for informational purposes. Inclusion or exclusion of a procedure, diagnosis or device code(s) does not constitute or imply member coverage or provider reimbursement policy. Please refer to the member's contract benefits in effect at the time of service to determine coverage or non-coverage of these services as it applies to an individual member.

HCPCS

J1458	Injection, galsulfase, 1 mg [Naglazyme]
S9357	Home infusion therapy, enzyme replacement intravenous therapy, (e.g., Imiglucerase); administrative services, professional pharmacy services, care coordination, and all necessary supplies and equipment (drugs and nursing visits coded separately), per diem

ICD-10 Diagnosis

E76.29	Other mucopolysaccharidoses (includes Maroteaux-Lamy syndrome)
E76.3	Mucopolysaccharidosis, unspecified

Document History

Reviewed: 9/9/2024
Document History:

- 9/9/2024 – Annual Review: No changes. Coding Reviewed: No changes.
- 9/11/2023 – Annual Review: Wording and formatting changes. Coding Reviewed: No changes.
- 9/12/2022 – Annual Review: No changes. Coding Reviewed: No changes.
- 9/13/2021 – Annual Review: Wording and formatting changes. Coding reviewed: No changes.
- 08/01/2021 – Administrative update to add documentation.
- 09/14/2020 – Annual Review: Addition of continuation criteria. Coding Reviewed: No changes.
- 09/23/2019 – Administrative update to add drug specific quantity limit.
- 09/9/2019 – Annual Review: Wording and formatting changes. Reference update. Coding reviewed: Added ICD-10-CM E76.3.
- 08/17/2018 – Annual Review: Updated diagnostic criteria for consistency with other CG for MPS by adding language to the enzyme activity criteria to rule out multiple sulfatase deficiency and also adding genetic testing as an option to confirm diagnosis. Add references for non-label based criteria elements.

References

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2. DailyMed. Package inserts. U.S. National Library of Medicine, National Institutes of Health website. http://dailymed.nlm.nih.gov/dailymed/about.cfm. Accessed: September 7, 2024.
3. DrugPoints® System [electronic version]. Truven Health Analytics, Greenwood Village, CO. Updated periodically.
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.
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6. Valayannopoulos V, Nicely H, Harmatz P, Turbeville S. Mucopolysaccharidosis VI. *Orphanet J Rare Dis*. 2010; 5:5.
7. Wang RY, Bodamer OA, Watson MS, Wilcox WR. American College of Medical Genetics (ACMG) Work Group on Diagnostic Confirmation of Lysosomal Storage Diseases. Lysosomal storage diseases: diagnostic confirmation and management of presymptomatic individuals. *Genet Med*. 2011; 13(5):457-484.
8. 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.

Federal and state laws or requirements, contract language, and Plan utilization management programs or polices may take precedence over the application of this clinical criteria.