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Coverage Policy Number **IP0443**

Galsulfase

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Related Coverage Resources

INSTRUCTIONS FOR USE
The following Coverage Policy applies to health benefit plans administered by Cigna Companies. Certain Cigna Companies and/or lines of business only provide utilization review services to clients and do not make coverage determinations. References to standard benefit plan language and coverage determinations do not apply to those clients. Coverage Policies are intended to provide guidance in interpreting certain standard benefit plans administered by Cigna Companies. Please note, the terms of a customer's particular benefit plan document [Group Service Agreement, Evidence of Coverage, Certificate of Coverage, Summary Plan Description (SPD) or similar plan document] may differ significantly from the standard benefit plans upon which these Coverage Policies are based. For example, a customer's benefit plan document may contain a specific exclusion related to a topic addressed in a Coverage Policy. In the event of a conflict, a customer's benefit plan document always supersedes the information in the Coverage Policies. In the absence of a controlling federal or state coverage mandate, benefits are ultimately determined by the terms of the applicable benefit plan document. Coverage determinations in each specific instance require consideration of 1) the terms of the applicable benefit plan document in effect on the date of service; 2) any applicable laws/regulations; 3) any relevant collateral source materials including Coverage Policies and; 4) the specific facts of the particular situation. Coverage Policies relate exclusively to the administration of health benefit plans. Coverage Policies are not recommendations for treatment and should never be used as treatment guidelines. In certain markets, delegated vendor guidelines may be used to support medical necessity and other coverage determinations.

Overview

This policy supports medical necessity review for galsulfase intravenous infusion (**Naglazyme**®).

Receipt of sample product does not satisfy any criteria requirements for coverage.

Medical Necessity Criteria

Galsulfase (Naglazyme) is considered medically necessary when the following are met:

1. **Mucopolysaccharidosis Type VI (MPS VI, Maroteaux-4 Lamy Syndrome).** Individual meets **BOTH** of the following criteria (A and B):
    A. The diagnosis is established by **ONE** of the following (i or ii):
        i. Individual has a laboratory test demonstrating deficient N-acetylgalactosamine 4-sulfatase (arylsulfatase B) activity in leukocytes, fibroblasts, or dried blood spots

ii. Individual has a molecular genetic test demonstrating biallelic pathogenic or likely pathogenic arylsulfatase B (*ARSB*) gene variants

B. The medication is prescribed by, or in consultation with, a geneticist, endocrinologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of lysosomal storage disorders.

When coverage is available and medically necessary, the dosage, frequency, duration of therapy, and site of care should be reasonable, clinically appropriate, and supported by evidence-based literature and adjusted based upon severity, alternative available treatments, and previous response to therapy.

Reauthorization Criteria

Galsulfase (Naglazyme) is considered medically necessary for continued use when initial criteria are met AND there is documentation of beneficial response.

Authorization Duration

Initial and reauthorization approval duration: up to 12 months

Conditions Not Covered

Any other use is considered experimental, investigational, or unproven.

Coding Information

Note:

1) This list of codes may not be all-inclusive.
2) Deleted codes and codes which are not effective at the time the service is rendered may not be eligible for reimbursement.

Considered Medically Necessary when criteria in the applicable policy statements listed above are met:

HCPCS Codes	Description
J1458	Injection, galsulfase, 1 mg

Background

OVERVIEW

Naglazyme, a human *N*-acetylgalactosamine 4-sulfatase, is indicated for patients with **Mucopolysaccharidosis type VI** (Maroteaux – Lamy syndrome [MPS VI]).[1] It is produced in a Chinese hamster ovary cell line via recombinant DNA technology. The enzyme catalyzes the hydrolysis of the sulfate ester from the glycosaminoglycans, chondroitin 4-sulfate and dermatan sulfate. Naglazyme has been shown to improve walking and stair climbing capacity.

Disease Overview

MPS VI, or Maroteaux – Lamy syndrome, is a rare lysosomal storage disorder characterized by a deficiency of *N*-acetylgalactosamine 4-sulfatase (arylsulfatase B).[2,3] The enzyme deficiency results in the accumulation of partially hydrolyzed dermatan sulfate and chondroitin 4-sulfate in lysosomes leading to the signs and symptoms of the disease.[2,3] The onset, severity and rate of progression of MPS VI is heterogeneous; however, most patients are severely affected with a rapidly progressive form.[3] Clinical manifestations include course facial features, short stature, kyphoscoliosis, joint stiffness, pulmonary insufficiency, cardiac disease, hepatosplenomegaly, corneal clouding, and hernias.[2,3] The definitive diagnosis of MPS VI is established by demonstrating deficient arylsulfatase B enzyme activity in leukocytes or fibroblasts, or by genetic testing.[2,3] Definitive treatment of MPS VI consists of

either enzyme replacement therapy with Naglazyme or hematopoietic stem cell transplantation. Due to the morbidity and mortality associated with hematopoietic stem cell transplantation, this therapy is typically reserved for patients who are intolerant of or do not respond to enzyme replacement therapy.[2]

Dosing and Availability
The recommended dosage is 1 mg per kg of body weight administered once weekly as an intravenous infusion.
Naglazyme 5 mg/5 mL single-dose vials are supplied as a colorless to pale yellow, clear to slightly opalescent solution.

References

1. Naglazyme® intravenous infusion [prescribing information]. Novato, CA: BioMarin; April 2020.
2. Harmatz PR, Shediac R. Mucopolysaccharidosis VI: Pathophysiology, diagnosis and treatment. *Front Biosci*. 2017;22:385-406.
3. Vairo F, Federhen A, Baldo G, et al. Diagnostic and treatment strategies in mucopolysaccharidosis VI. *Appl Clin Genet*. 2015;8:245-255.