Clinical Policy: Galsulfase (Naglazyme)

Reference Number: PA.CP.PHAR.161
Effective Date: 01/18
Last Review Date: 04/18

Coding Implications
Revision Log

Description
The intent of the criteria is to ensure that patients follow selection elements established by Pennsylvania Health and Wellness® clinical policy for galsulfase (Naglazyme®)

FDA approved indication
Naglazyme is indicated for the treatment of patients with mucopolysaccharidosis VI (MPS VI; Maroteaux-Lamy syndrome). Naglazyme has been shown to improve walking and stair-climbing capacity.

Policy/Criteria
It is the policy of Pennsylvania Health and Wellness that Naglazyme is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Maroteaux-Lamy Syndrome (Mucopolysaccharidosis VI [MPS VI]): (must meet all):

1. Diagnosis of Maroteaux-Lamy syndrome (MPS VI) confirmed by one of the following:
   a. Enzyme assay demonstrating a deficiency in N-acetygalactosamine 4-sulfatase (arylsulfatase B) activity;
2. DNA testing.
3. Age ≥ 3 months;
4. Dose does not exceed 1 mg/kg/week.
   a.

Approval duration: 6 months

B. Other diagnoses/indications: Refer to PA.CP.PHAR.57 - Global Biopharm Policy.

II. Continued Approval

A. Maroteaux-Lam Syndrome (MPS VI): (must meet all)**:**

1. Currently receiving medication via Pennsylvania Health and Wellness benefit or member has previously met all initial approval criteria; or the Continuity of Care policy (PA.LTSS.PHAR.01) applies;
1. Member is responding positively to therapy as evidenced by improvement in the individual member's MPS VI (Maroteaux-Lamy syndrome) manifestation profile *(see Appendix C for examples)*;
2. If request is for a dose increase, new dose does not exceed 1 mg/kg/week.

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2)**:**
1. Currently receiving medication via Pennsylvania Health and Wellness benefit and documentation supports positive response to therapy; or the Continuity of Care policy (PA.LTSS.PHAR.01) applies; or
2. Refer to PA.CP.PHAR.57 - Global Biopharm Policy**.**

Background
Description/Mechanism of Action:
Mucopolysaccharide storage disorders are caused by the deficiency of specific lysosomal enzymes required for the catabolism of glycosaminoglycans (GAG). MPS VI is characterized by the absence or marked reduction in N–acetylgalactosamine 4-sulfatase. The sulfatase activity deficiency results in the accumulation of the GAG substrate, dermatan sulfate, throughout the body. This accumulation leads to widespread cellular, tissue, and organ dysfunction. Naglazyme is intended to provide an exogenous enzyme that will be taken up into lysosomes and increase the catabolism of GAG. Galsulfase uptake by cells into lysosomes is most likely mediated by the binding of mannose-6-phosphate-terminated oligosaccharide chains of galsulfase to specific mannose-6-phosphate receptors.

Formulations:
Naglazyme (galsulfase): Solution for reconstitution; for intravenous use
- 5 mg/5 mL vial; 1 mg/mL (70 units/mg)

Appendices
Appendix A: Abbreviation Key
GAG: Glycosaminoglycan
MPS: Mucopolysaccharide

Appendix B: Therapeutic Alternatives
Not applicable

Appendix C: General Information
The presenting symptoms and clinical course of MPS VI can vary from one individual to another. Some examples, however, of improvement in MPS VI disease as a result of Naglazyme therapy may include improvement in:
- 12-minute walking test distance;
- 3-minute stair climb rate;
- Poor endurance;
- Vision problems;
- Respiratory infections;
- Breathing problems, sleep apnea;
- High blood pressure;
- Joint stiffness;
- Hepatomegaly, splenomegaly.

Coding Implications
Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J1458	Injection, galsulfase, 1 mg

Reviews, Revisions, and Approvals	Date	Approval Date
2Q 2018 annual review: Modified age restriction to 3 months per PI. Added prescriber requirement. Added max dose criteria. Added requirement for positive response to therapy. R eferences reviewed and updated.	02.13.18	04.18

References
1. Naglazyme prescribing information. Novato, CA: BioMarin Pharmaceutical, Inc.; March 2013. Available at http://www.naglazyme.com. Accessed February 26, 2018.
2. Muenzer J. The mucopolysaccharidoses: A heterogeneous group of disorders with variable pediatric presentations. J Pediatr. 2004; 144(5 Suppl): S27-S34.