Clinical Policy: Galsulfase (Naglazyme)

Reference Number: ERX.SPA.101
Effective Date: 10.01.16
Last Review Date: 05.20
Line of Business: Commercial, Medicaid

Revision Log

See Important Reminder at the end of this policy for important regulatory and legal information.

Description
Galsulfase (Naglazyme®) is a hydrolytic lysosomal glycosaminoglycan-specific enzyme.

FDA Approved Indication(s)
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
Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

Health plan approved formularies should be reviewed for all coverage determinations. Requirements to use preferred alternative agents apply only when such requirements align with the health plan approved formulary.

It is the policy of health plans affiliated with Envolve Pharmacy Solutions™ that Naglazyme is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Mucopolysaccharidosis VI: Maroteaux-Lamy Syndrome (must meet all)**:**

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

Approval duration: 6 months

B. Other diagnoses/indications

1. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

II. Continued Therapy

A. Mucopolysaccharidosis VI: Maroteaux-Lamy Syndrome (must meet all)**:**

1. Currently receiving medication via a health plan affiliated with Envolve Pharmacy Solutions or member has previously met initial approval criteria;
2. Member is responding positively to therapy as evidenced by improvement in the individual member's MPS VI (Maroteaux-Lamy syndrome) manifestation profile *(see Appendix D for examples)*;
3. If request is for a dose increase, new dose does not exceed 1 mg per kg per week.

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2)**:**

1. Currently receiving medication via a health plan affiliated with Envolve Pharmacy Solutions and documentation supports positive response to therapy.
   Approval duration: Duration of request or 6 months (whichever is less); or
2. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

III. Diagnoses/Indications for which coverage is NOT authorized:

A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off-label use policy – ERX.PA.01 or evidence of coverage documents.

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key
FDA: Food and Drug Administration
MPS VI: mucopolysaccharidosis VI
12MWT: 12-minute walking test

Appendix B: Therapeutic Alternatives
Not applicable

Appendix C: Contraindications/Boxed Warnings
None reported

Appendix D: General Information

- In the clinical trial of Naglazyme in patients ≥ 5 years of age, patients treated with Naglazyme demonstrated an 83 meter mean increase relative to placebo in the 12-minute walking test (12MWT) and a mean improvement in the 3-minute stair climb rate of 4.7 stairs/minute relative to placebo, after 24 weeks of therapy.
- 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:
  - 12MWT distance;
  - 3-minute stair climb rate;
  - Poor endurance;
  - Vision problems;
  - Respiratory infections;
  - Breathing problems, sleep apnea;
  - High blood pressure;
  - Joint stiffness;
  - Height and weight;
  - Hepatomegaly, splenomegaly.

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
MPS VI	1 mg/kg IV once weekly	1 mg/kg/week

VI. Product Availability

Vial: 5 mg/5 mL

VII. References

1. Naglazyme Prescribing Information. Novato, CA: BioMarin Pharmaceutical, Inc.; December 2019. Available at http://www.naglazyme.com. Accessed February 20, 2020.
2. Muenzer J. The mucopolysaccharidoses: a heterogeneous group of disorders with variable pediatric presentations. J Pediatr. 2004; 144(5 Suppl): S27-S34.
3. Akyol MU, Alden TD, Amartino H, et al. Recommendations for the management of MPS VI: systematic evidence- and consensus-based guidance. Orphanet J of Rare Dis, 2019;12(118)1-21.

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy split from USS.SPMN.33 Lysosomal Storage Disorders and converted to new template. Added age restriction per PI. Modified approval duration to 6 months for initial and 12 months for re-auth.	08.16	09.16
Converted to new template. Modified age restriction to 3 months per PI. Added prescriber requirement. Added max dose criteria. Added requirement for positive response to therapy.	06.17	08.17
4Q17 Annual Review Removed prescriber requirement.	09.11.17	11.17
2Q 2018 annual review: No significant changes. References reviewed and updated.	02.26.18	05.18
2Q 2019 annual review: no significant changes; references reviewed and updated.	02.28.19	05.19
2Q 2020 annual review: no significant changes; references reviewed and updated.	02.20.20	05.20

Important Reminder

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information.

This Clinical Policy is not intended to dictate to providers how to practice medicine, nor does it constitute a contract or guarantee regarding payment or results. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members.