Medical Drug Clinical Criteria

Subject:	Vimizim (elosulfase alfa)		
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Table of Contents

Overview | Coding | References

Clinical criteria | Document history

Overview

This document addresses Vimizim (elosulfase alfa), an enzyme replacement therapy approved by the Food and Drug Administration (FDA) to treat individuals with Mucopolysaccharidosis IVA (Morquio A 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.

Vimizim has a black box warning for anaphylaxis. Life-threatening anaphylactic reactions have occurred during Vimizim infusions. Appropriate medical support should be available during Vimizim administration. Individuals should be educated on the signs and symptoms of anaphylaxis and to seek immediate medical care should they occur. Individuals with acute respiratory illness may be at risk of serious acute exacerbation of their respiratory disease and require additional monitoring.

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.

Vimizim (elosulfase alfa)

Initial requests for Vimizim (elosulfase alfa) may be approved if the following criteria are met:

I. Individual has a diagnosis of mucopolysaccharidosis IVA (Morquio A syndrome); **AND**
II. Documentation is provided that diagnosis is demonstrated by (Akyol 2019, Wood 2013**)**:
    A. Documented reduced fibroblast or leukocyte N-acetylgalactosamine-6-sulfatase (GALNS) enzyme activity *combined with* normal enzyme activity level of another sulfatase; **OR**
    B. Documented *GALNS* genetic mutation; **AND**
III. Individual demonstrates clinical signs and symptoms of Morquio A syndrome (for example, knee deformity, corneal opacity or pectus carinatum) (Hendriksz 2015, Wood 2013)**.**

Continuation requests for Vimizim (elosulfase alfa) 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.

Requests for Vimizim (elosulfase alfa) may not be approved for the following:

I. Individual is using to treat mucopolysaccharidosis IVB (Morquio B syndrome); **OR**
II. May not be approved when the above criteria are not met and for all other indications.

Quantity Limits

Vimizim (elosulfase alfa) Quantity Limit

Drug	Limit
Vimizim (elosulfase alfa) 5 mg vial	2 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

J1322	Injection, elosulfase alfa, 1 mg [Vimizim]
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

E76.210	Morquio A mucopolysaccharidoses

Document History

Reviewed: 9/9/2024
Document History:

- 9/9/2024 – Annual Review: No changes. Coding Reviewed: No changes.
- 9/11/2023 – Annual Review: Clarify diagnosis confirmation criteria. Wording and formatting changes. Coding Reviewed: No changes.
- 9/12/2022 – Annual Review: Wording and formatting changes. Coding Reviewed: Added ICD-10-CM E76.210. Remove ICD-10-CM E76.210-E76.219.
- 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 Review: 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: No Changes
- 08/17/2018 – Annual Review: Update diagnostic criteria for consistency with other CG for MPS by adding language to the enzyme activity criteria to rule out multiple sulfatase deficiency. Add references for non-label based criteria elements. Wording and formatting updates.

References

1. Akyol MU, Alden TD, Amartino H, et al. Recommendations for the management of MPS IVA: systematic evidence and consensus-based guidance. *Orphanet J Rare Dis*. 2019;14(1):137. doi: 10.1186/s13023-019-1074-9
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. Hendriksz CJ, Berger KI, Giugliani R, at al. International guidelines for the management and treatment of Morquio A syndrome. *Am J Med Genet A*. 2015; 167A(1):11-25.
5. 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.
6. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc. Updated periodically.

7. Wood TC, Harvey K, Beck M, et al. Diagnosing mucopolysaccharidosis IVA. *J Inherit Metab Dis*. 2013;36:293–307.

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.