# Pharmacy Medical Necessity Guidelines: Vimizim® (elosulfase alfa)

*Effective: June 15, 2020*

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|---|---|---|---|
| Prior Authorization Required | √ | Type of Review – Care Management | |
| Not Covered | | Type of Review – Clinical Review | √ |
| Pharmacy (RX) or Medical (MED) Benefit | MED | Department to Review | PRECERT /MM |

These pharmacy medical necessity guidelines apply to the following:

**Commercial Products**

☒ Tufts Health Plan Commercial products – large group plans
☒ Tufts Health Plan Commercial products – small group and individual plans
☒ Tufts Health Freedom Plan products – large group plans
☒ Tufts Health Freedom Plan products – small group plans
- CareLink℠ – Refer to CareLink Procedures, Services and Items Requiring Prior Authorization

**Tufts Health Public Plans Products**

☒ Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product)
☒ Tufts Health Together – MassHealth MCO Plan and Accountable Care Partnership Plans
☒ Tufts Health RITogether – A Rhode Island Medicaid Plan

**Fax Numbers:**

All plans except Tufts Health Public Plans:
PRECERT: 617.972.9409

Tufts Health Public Plans only:
MM: 888.415.9055

**Note:** This guideline does not apply to Medicare Members (includes dual eligible Members).

## OVERVIEW

**FOOD AND DRUG ADMINISTRATION-APPROVED INDICATIONS**

Vimizim (elosulfase alfa) is a hydrolytic lysosomal glycosaminoglycan (GAG)-specific enzyme indicated for patients with Mucopolysaccharidosis type IVA (MPS IVA; Morquio A syndrome) in adults and children 5 years of age and older.

MPS IVA is a very rare and debilitating genetic disorder which is caused by a deficiency of the enzyme, *N*-acetylgalactosamine-6 sulfatase, which results in excessive lysosomal storage of keratan sulfate in many tissues and organs. Accumulation of keratan sulfate causes systemic skeletal dysplasia, short stature, and joint abnormalities, which limit mobility and endurance. Malformation of thorax impairs respiratory function and malformation of neck vertebrae and ligament weakness causes cervical spinal instability and, potentially, cord compression. Other symptoms include hearing loss, corneal clouding, and heart valve disease.

## COVERAGE GUIDELINES

The plan may authorize coverage of Vimizim (elosulfase alfa) for Members when all the following criteria are met:

1. Documented diagnosis of Mucopolysaccharidosis type IV A (MPS IVA; Morquio A syndrome) disease by a specialist

**AND**

2. Member is at least 5 years of age

## LIMITATIONS

- The plan does NOT cover Vimizim (elosulfase alfa) for the treatment of Mucopolysaccharidosis IV B.

## CODES

The following HCPCS/CPT code(s) are:

| Code | Description |
|---|---|
| J1322 | Injection, elosulfase alfa, 1 mg |

## REFERENCES


1. Hendriksz CJ, Burton B, Fleming TR, et al. Efficacy and safety of enzyme replacement therapy with BMN 110 (elosulfase alfa) for Morquio A syndrome (mucopolysaccharidosis IVA): a phase 3 randomised placebo-controlled study. *J Inherit Metab Dis*. 2014 Nov;37(6):979-90.
2. Hendriksz CJ, Giugliani R, Harmatz P, et al. Multi-domain impact of elosufase alfa in Morquio A syndrome in the pivotal phase III trial. *Mol Genet Metab*. 2015 Feb;114(2):178-85
3. Vimizim (elosulfase alfa) [prescribing information]. Novato, CA: BioMarin Pharmaceuticals Inc; 2014 February.

4. Wang RY, Bodamer OA, Watson MS et al. American College of Medical Genetics: Lysosomal Storage Diseases: Diagnostic Confirmation and Management of Presymptomatic Individuals. *Genetics in Medicine.* 2011; 13(5):464-466.
5. Wenger DA, Coppola S, Liu SL. Insights into the diagnosis and treatment of lysosomal storage diseases. *Archives of Neurology*. 2003; 60:322-8.
6. Wyatt K, Henley W, Anderson L et al. The effectiveness and cost-effectiveness of enzyme and substrate replacement therapies: a longitudinal cohort study of people with lysosomal storage disorders. *Health Technol Assess.* 2012; 16(39):1-543.

## APPROVAL HISTORY

June 10, 2014: Reviewed by Pharmacy & Therapeutics Committee.

Subsequent endorsement date(s) and changes made:
1. July 1, 2014: Administrative Update: Added HCPCS code to Medical Necessity Guideline.
2. June 9, 2015: No changes
3. October 30, 2015: Administrative Update: Replaced procedural code C9022 with J1322.
4. January 1, 2016: Administrative change to rebranded template.
5. June 14, 2016: No changes
6. April 11, 2017: Administrative update. Effective 6/1/2017, Medical Necessity Guideline applies to Tufts Health RITogether.
7. June 13, 2017: No changes
8. July 10, 2018: No changes
9. August 13, 2019: No changes
10. June 9, 2020: Removed the reauthorization criteria and documentation of a baseline 6-minute walk test. Administrative update to move age requirements from Limitations section to Coverage Guidelines.

## BACKGROUND, PRODUCT AND DISCLAIMER INFORMATION

Pharmacy Medical Necessity Guidelines have been developed for determining coverage for plan benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. The plan makes coverage decisions on a case-by-case basis considering the individual member's health care needs. Pharmacy Medical Necessity Guidelines are developed for selected therapeutic classes or drugs found to be safe, but proven to be effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in the service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. The plan revises and updates Pharmacy Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

For self-insured plans, coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a Pharmacy Medical Necessity Guideline and a self-insured Member's benefit document, the provisions of the benefit document will govern.

Treating providers are solely responsible for the medical advice and treatment of members. The use of this policy is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to member eligibility and benefits on the date of service, coordination of benefits, referral/authorization and utilization management guidelines when applicable, and adherence to plan policies and procedures and claims editing logic.