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5.30.023

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| **Section:** | Prescription Drugs | **Effective Date:** | April 1, 2025 |
| **Subsection:** | Endocrine and Metabolic Drugs | **Original Policy Date:** | October 1, 2014 |
| **Subject:** | Vimizim | **Page:** | 1 of 4 |

***Last Review Date:*** March 7, 2025

# Vimizim

## Description

### Vimizim (elosulfase alfa)

**Background**
Vimizim is an enzyme used to treat patients with Mucopolysaccharidosis Type IVA (Morquio A syndrome). Morquio A syndrome is a rare autosomal recessive lysosomal storage disease caused by a deficiency in N-acetylgalactosamine-6-sulfate sulfatase (GALNS). Vimizim is intended to replace the missing GALNS enzyme involved in an important metabolic pathway. Absence of this enzyme leads to problems with bone development, growth, and mobility (1).

**Regulatory Status**
FDA-approved indication: Vimizim is a hydrolytic lysosomal glycosaminoglycan (GAG)-specific enzyme indicated for patients with Mucopolysaccharidosis type IVA (MPS IVA; Morquio A syndrome) (1).

Life-threatening anaphylactic reactions have occurred in some patients during Vimizim infusions. Patients with compromised respiratory function or acute respiratory disease may be at risk of serious acute exacerbation of their respiratory compromise due to infusion reactions and require additional monitoring. Appropriate medical support should be readily available when Vimizim is administered. Closely observe patients during and after Vimizim administration and be prepared to manage anaphylaxis. Inform patients of the signs and symptoms of anaphylaxis and have them seek immediate medical care should symptoms occur (1).

Sleep apnea is common in MPS IVA patients. Evaluation of airway patency should be considered prior to initiation of treatment with Vimizim. Patients using supplemental oxygen or

continuous positive airway pressure (CPAP) during sleep should have these treatments readily available during infusion in the event of an acute reaction, or extreme drowsiness/sleep induced by antihistamine use (1).

Safety and effectiveness in patients below 5 years of age have not been established (1).

**Related policies**
Aldurazyme, Elaprase, Mepsevii, Naglazyme

## Policy

*This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.*

Vimizim may be considered **medically necessary** if the conditions indicated below are met.

Vimizim may be considered **investigational** for all other indications.

## Prior-Approval Requirements

**Age** 5 years of age or older

**Diagnosis**

Patient must have the following:

Mucopolysaccharidosis Type IVA (MPS IVA) (Morquio A syndrome)

**AND** at least **ONE** of the following:

1. Documented signs and symptoms of MPS IVA such as skeletal abnormalities and keratin sulfate levels in urine
2. Genetic testing confirming diagnosis of MPS IVA

## Prior – Approval *Renewal* Requirements

**Age** 5 years of age or older

**Diagnosis**

Patient must have the following:

Mucopolysaccharidosis Type IVA (MPS IVA) (Morquio A syndrome)

## Policy Guidelines

### Pre - PA Allowance

None

### Prior - Approval Limits

**Duration** 2 years

### Prior – Approval *Renewal* Limits

Same as above

## Rationale

**Summary**

Vimizim is an enzyme preparation for patients diagnosed with Mucopolysaccharidosis type IVA (MPS IVA). Vimizim is intended to replace the missing GALNS enzyme involved in an important metabolic pathway. Anaphylaxis has been reported to occur during Vimizim infusions, regardless of duration of the course of treatment. Patients with compromised respiratory function or acute respiratory disease may be at risk of serious acute exacerbation of their respiratory compromise due to infusion reactions and require additional monitoring. Appropriate medical support should be readily available when Vimizim is administered. Safety and efficacy have not been established in pediatric patients less than five years of age (1).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Vimizim while maintaining optimal therapeutic outcomes.

**References**

1. Vimizim [package insert]. Novato, CA: BioMarin Pharmaceutical Inc.; December 2019.

## Policy History

| Date | Action |
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| September 2014 | PMPC review |
| October 2014 | New addition to PA |
| December 2015 | Annual review |
| September 2016 | Annual editorial review and reference update<br>Policy number change from 5.08.18 to 5.30.23 |
| December 2017 | Annual editorial review |
| June 2018 | Annual editorial review |
| December 2019 | Annual editorial review. Changed approval duration from lifetime to 2 years |
| December 2020 | Annual review and reference update |
| December 2021 | Annual review |
| December 2022 | Annual review. Changed policy number to 5.30.023 |
| March 2023 | Annual review |
| March 2024 | Annual review |
| March 2025 | Annual review |

## Keywords

**This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 7, 2025 and is effective on April 1, 2025.**