



5.30.023

Section:	Prescription Drugs	Effective Date:	April 1, 2024
Subsection:	Endocrine and Metabolic Drugs	Original Policy Date:	October 1, 2014
Subject:	Vimizim	Page:	1 of 4

Last Review Date: March 8, 2024

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

Section:	Prescription Drugs	Effective Date:	April 1, 2024
Subsection:	Endocrine and Metabolic Drugs	Original Policy Date:	October 1, 2014
Subject:	Vimizim	Page:	2 of 4

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

Section:	Prescription Drugs	Effective Date:	April 1, 2024
Subsection:	Endocrine and Metabolic Drugs	Original Policy Date:	October 1, 2014
Subject:	Vimizim	Page:	3 of 4

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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5.30.023

Section:	Prescription Drugs	Effective Date:	April 1, 2024
Subsection:	Endocrine and Metabolic Drugs	Original Policy Date:	October 1, 2014
Subject:	Vimizim	Page:	4 of 4

September 2014	PMPC review
October 2014	New addition to PA
December 2015	Annual review
September 2016	Annual editorial review and reference update 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

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 8, 2024 and is effective on April 1, 2024.