Reference number(s)
4205-A

Standard Medicare Part B Management Vimizim

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Vimizim	elosulfase alfa

Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-approved Indications[1]

Vimizim is indicated for patients with Mucopolysaccharidosis type IVA (MPS IVA, Morquio A syndrome).

All other indications will be assessed on an individual basis. Submissions for indications other than those listed in this criteria should be accompanied by supporting evidence from Medicare approved compendia.

Documentation

The following documentation must be available, upon request, for all submissions:

Initial Requests:

- N-acetylgalactosamine-6-sulfatase enzyme assay or genetic testing results supporting diagnosis.

Continuation Requests:

© 2026 CVS Caremark. All rights reserved.

This document contains confidential and proprietary information of CVS Caremark and cannot be reproduced, distributed or printed without written permission from CVS Caremark. This document contains prescription brand name drugs that are trademarks or registered trademarks of pharmaceutical manufacturers that are not affiliated with CVS Caremark.

- Chart notes or medical records documenting a clinically positive response to therapy, which shall include improvement, stabilization, or slowing of disease progression (e.g., improvement, stabilization, or slowing of disease progression for 6-minute walk test, 3-minute stair climb, urine keratan sulfate levels).

Coverage Criteria

Mucopolysaccharidosis IVA (MPS IVA, Morquio A syndrome)[1,2]

Authorization of 12 months may be granted for treatment of MPS IVA (Morquio A syndrome) when the diagnosis of MPS IVA was confirmed by enzyme assay demonstrating a deficiency of N-acetylgalactosamine-6-sulfatase enzyme activity or by genetic testing.

Continuation of Therapy

All members (including new members) requesting authorization for continuation of therapy must be currently receiving therapy with the requested agent.

Authorization of 12 months may be granted when all of the following criteria are met:

- The member is currently receiving therapy with the requested medication.
- The requested medication is being used to treat an indication listed in the Coverage Criteria section.
- The member is receiving benefit from therapy. Benefit is defined as a clinically positive response to therapy, which shall include improvement, stabilization, or slowing of disease progression (e.g., improvement, stabilization, or slowing of disease progression for 6-minute walk test, 3-minute stair climb, urine keratan sulfate levels).

Summary of Evidence

The contents of this policy were created after examining the following resources:

- The prescribing information for Vimizim.
- The available compendium
    - National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium
    - Micromedex DrugDex
    - American Hospital Formulary Service- Drug Information (AHFS-DI)
    - Lexi-Drugs
    - Clinical Pharmacology
- International guidelines for the management and treatment of Morquio A syndrome.

After reviewing the information in the above resources, the FDA-approved indications listed in the prescribing information for Vimizim are covered.

© 2026 CVS Caremark. All rights reserved.

This document contains confidential and proprietary information of CVS Caremark and cannot be reproduced, distributed or printed without written permission from CVS Caremark. This document contains prescription brand name drugs that are trademarks or registered trademarks of pharmaceutical manufacturers that are not affiliated with CVS Caremark.

Explanation of Rationale

Support for FDA-approved indications can be found in the manufacturer's prescribing information.

Support for confirming the diagnosis with enzyme assays or genetic testing prior to using Vimizim to treat MPS IVA can be found in the guidelines published by Hendriksz et al. A definite diagnosis entails demonstration of reduced N-acetylgalactosamine-6-sulfatase activity in leukocytes or fibroblasts. Molecular analysis can be performed as a further confirmation of the diagnosis.

References

1. Vimizim [package insert]. Novato, CA: BioMarin Pharmaceutical Inc.; December 2019.
2. Hendriksz CJ, Berger KI, Giugliani R, et al. International guidelines for the management and treatment of Morquio A syndrome. Am J Med Genet A. 2015;167A(1):11-25.
3. Regier DS, Oetgen M, Tanpaiboon P. Mucopolysaccharidosis Type IVA. 2013 Jul 11 [Updated 2021 Jun 17]. In: Adam MP, Bick S, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2026.

© 2026 CVS Caremark. All rights reserved.

This document contains confidential and proprietary information of CVS Caremark and cannot be reproduced, distributed or printed without written permission from CVS Caremark. This document contains prescription brand name drugs that are trademarks or registered trademarks of pharmaceutical manufacturers that are not affiliated with CVS Caremark.