Reference number(s)
2057-A

Specialty Guideline 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 are considered experimental/investigational and not medically necessary.

Documentation

Submission of the following information is necessary to initiate the prior authorization review:

Initial Requests:

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

- Chart notes or medical records documenting clinical signs and symptoms of disease at baseline (e.g., kyphoscoliosis, genu valgum, pectus carinatum, respiratory compromise, valvular heart disease, hearing impairment, corneal clouding, hepatomegaly, neurologic impairment).

Continuation Requests:

- Chart notes 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).

Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of metabolic disease and/or lysosomal storage disorders.

Coverage Criteria

Mucopolysaccharidosis IVA (MPS IVA, Morquio A syndrome)[1-3]

Authorization of 12 months may be granted for treatment of MPS IVA (Morquio A syndrome) when both of the following criteria are met:

- The diagnosis of MPS IVA was confirmed by enzyme assay demonstrating a deficiency of N-acetylgalactosamine-6-sulfatase enzyme activity or by genetic testing.
- Member exhibits clinical signs and symptoms of disease at baseline (e.g., kyphoscoliosis, genu valgum, pectus carinatum, respiratory compromise, valvular heart disease, hearing impairment, corneal clouding, hepatomegaly, neurologic impairment).

Continuation of Therapy

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in the Coverage Criteria section who have 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).

Reference number(s)
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References

1. Vimizim [package insert]. Novato, CA: BioMarin Pharmaceutical Inc.; October 2025.
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.