

STANDARD MEDICARE PART B MANAGEMENT

VIMIZIM (elosulfase alfa)

POLICY

I. 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 Indication

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 enumerated in this policy should be accompanied by supporting evidence from Medicare approved compendia.

II. DOCUMENTATION

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

- A. Initial requests: N-acetylgalactosamine 6-sulfatase enzyme assay or genetic testing results supporting diagnosis.
- B. Continuation requests: chart notes documenting a clinically positive response to therapy, which shall include improvement, stabilization, or slowing of disease progression.

III. CRITERIA FOR INITIAL APPROVAL

Mucopolysaccharidosis IVA (MPS IVA)

Authorization of 12 months may be granted for treatment of MPS IVA 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.

IV. 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:

- A. The member is currently receiving therapy with Vimizim
- B. Vimizim is being used to treat an indication enumerated in Section III

- C. 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.

V. SUMMARY OF EVIDENCE

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

1. The prescribing information for Vimizim.
2. The available compendium
 - a. National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium
 - b. Micromedex DrugDex
 - c. American Hospital Formulary Service- Drug Information (AHFS-DI)
 - d. Lexi-Drugs
 - e. Clinical Pharmacology
3. 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.

VI. 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.

VII. 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.