



## Drug Coverage Policy

Effective Date.....07/15/2025

Coverage Policy Number ..... IP0442

Policy Title.....Vimizim

## Enzyme Replacement Therapy – Vimizim

- Vimizim® (elosulfase alfa intravenous infusion – BioMarin)

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### INSTRUCTIONS FOR USE

*The following Coverage Policy applies to health benefit plans administered by Cigna Companies. Certain Cigna Companies and/or lines of business only provide utilization review services to clients and do not make coverage determinations. References to standard benefit plan language and coverage determinations do not apply to those clients. Coverage Policies are intended to provide guidance in interpreting certain standard benefit plans administered by Cigna Companies. Please note, the terms of a customer's particular benefit plan document [Group Service Agreement, Evidence of Coverage, Certificate of Coverage, Summary Plan Description (SPD) or similar plan document] may differ significantly from the standard benefit plans upon which these Coverage Policies are based. For example, a customer's benefit plan document may contain a specific exclusion related to a topic addressed in a Coverage Policy. In the event of a conflict, a customer's benefit plan document always supersedes the information in the Coverage Policies. In the absence of a controlling federal or state coverage mandate, benefits are ultimately determined by the terms of the applicable benefit plan document. Coverage determinations in each specific instance require consideration of 1) the terms of the applicable benefit plan document in effect on the date of service; 2) any applicable laws/regulations; 3) any relevant collateral source materials including Coverage Policies and; 4) the specific facts of the particular situation. Each coverage request should be reviewed on its own merits. Medical directors are expected to exercise clinical judgment where appropriate and have discretion in making individual coverage determinations. Where coverage for care or services does not depend on specific circumstances, reimbursement will only be provided if a requested service(s) is submitted in accordance with the relevant criteria outlined in the applicable Coverage Policy, including covered diagnosis and/or procedure code(s). Reimbursement is not allowed for services when billed for conditions or diagnoses that are not covered under this Coverage Policy (see "Coding Information" below). When billing, providers must use the most appropriate codes as of the effective date of the submission. Claims submitted for services that are not accompanied by covered code(s) under the applicable Coverage Policy will be denied as not covered. Coverage Policies relate exclusively to the administration of health benefit plans. Coverage Policies are not recommendations for treatment and should never be used as treatment guidelines. In certain markets, delegated vendor guidelines may be used to support medical necessity and other coverage determinations.*

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## OVERVIEW

Vimizim, a human *N*-acetylgalactosamine-6-sulfatase, is indicated for **Mucopolysaccharidosis type IVA** (Morquio A syndrome [MPS IVA]).<sup>1</sup> It is produced in Chinese hamster ovary cells via recombinant DNA technology. Vimizim is a hydrolytic lysosomal enzyme which is taken up by lysosomes and hydrolyzes sulfate from the non-reduced ends of the glycosaminoglycans keratan sulfate and chondroitin-6-sulfate.

## Disease Overview

MPS IVA (Morquio A syndrome) is a rare lysosomal storage disorder characterized by deficient *N*-acetylgalactosamine-6-sulfatase activity leading to the accumulation of chondroitin-6-sulfate and keratan sulfate in lysosomes in bone, cartilage, and ligaments.<sup>2,3</sup> The clinical course, onset, and severity of MPS IVA is heterogeneous.<sup>2</sup> Manifestations of MPS IVA include short trunk dwarfism with short neck, kyphoscoliosis, odontoid dysplasia, knock-knee, cervical spinal cord compression, hypermobile joints, cardiac disease, respiratory insufficiency, obstructive sleep apnea, corneal clouding, and dental abnormalities.<sup>2-4</sup> MPS IVA has not been associated with cognitive decline.<sup>2</sup> The definitive diagnosis of MPS IVA is established by demonstrating deficient *N*-acetylgalactosamine-6-sulfatase activity in leukocytes or fibroblasts; or by genetic testing.<sup>2</sup> Definitive treatment for MPS IVA consists of enzyme replacement therapy with Vimizim. Hematopoietic stem cell transplantation is not recommended for MPS IVA.

## Coverage Policy

### POLICY STATEMENT

**Prior Authorization is required for benefit coverage of Vimizim. Approval is recommended for those who meet the Criteria and Dosing for the listed indication. Extended approvals are allowed if the patient continues to meet the Criteria and Dosing. Requests for doses outside of the established dosing documented in this policy will be considered on a case-by-case basis by a clinician (i.e., Medical Director or Pharmacist). All approvals are provided for the duration noted below. Because of the specialized skills required for evaluation and diagnosis of patients treated with Vimizim as well as the monitoring required for adverse events and long-term efficacy, approval requires Vimizim to be prescribed by or in consultation with a physician who specializes in the condition being treated.**

**Vimizim is considered medically necessary when the following criteria are met:**

### FDA-Approved Indication

- 1. Mucopolysaccharidosis Type IVA (Morquio A Syndrome).** Approve for 1 year if the patient meets BOTH of the following (A and B):
  - A)** The diagnosis is established by ONE of the following (i or ii):
    - i.** Patient has a laboratory test demonstrating deficient *N*-acetylgalactosamine-6-sulfatase activity in leukocytes or fibroblasts; OR
    - ii.** Patient has a molecular genetic test demonstrating biallelic pathogenic or likely pathogenic *N*-acetylgalactosamine-6-sulfatase (*GALNS*) gene variants; AND
  - B)** Vimizim is prescribed by or in consultation with a geneticist, endocrinologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of lysosomal storage disorders.

**Dosing.** Approve up to 2 mg/kg of body weight administered intravenously no more frequently than once a week.

When coverage is available and medically necessary, the dosage, frequency, duration of therapy, and site of care should be reasonable, clinically appropriate, and supported by evidence-based literature and adjusted based upon severity, alternative available treatments, and previous response to therapy.

Receipt of sample product does not satisfy any criteria requirements for coverage.

### Conditions Not Covered

**Vimizin for any other use is considered not medically necessary. Criteria will be updated as new published data are available.**

## Coding Information

**Note:** 1) This list of codes may not be all-inclusive.  
2) Deleted codes and codes which are not effective at the time the service is rendered may not be eligible for reimbursement.

**Considered Medically Necessary when criteria in the applicable policy statements listed above are met:**

HCPSC Codes	Description
J1322	Injection, elosulfase alfa, 1 mg

## References

1. Vimizim® intravenous infusion [prescribing information]. Novato, CA: BioMarin; December 2019.
2. Akyol MU, et al. MPS Consensus Programme Co-Chairs. Recommendations for the management of MPS IVA: systematic evidence- and consensus-based guidance. *Orphanet J Rare Dis*. 2019 Jun 13;14(1):137.
3. Tomatsu S, Yasuda E, Patel P, et al. Morquio A syndrome: Diagnosis and current and future therapies. *Pediatr Endocrinol Rev*. 2014;12:141-151.
4. Regier DS, Tanpaiboon P. Role of elosulfase alfa in mucopolysaccharidosis IVA. *Appl Clin Genet*. 2016;9:67-74.

## Revision Details

Type of Revision	Summary of Changes	Date
Annual Revision	<b>Updated</b> coverage policy title from <i>Elosulfase Alfa</i> to <i>Enzyme Replacement Therapy – Vimizim</i> .  <b>Mucopolysaccharidosis Type IVA (Morquio A Syndrome):</b> <b>Added</b> dosing.	8/1/2024
Annual Revision	No criteria changes	07/15/2025

The policy effective date is in force until updated or retired.

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