

## Clinical Policy: Elosulfase Alfa (Vimizim)

Reference Number: CP.PHAR.162

Effective Date: 02/16

Last Review Date: 02/17

[Coding Implications](#)  
[Revision Log](#)

See [Important Reminder](#) at the end of this policy for important regulatory and legal information.

### Description

The intent of the criteria is to ensure that patients follow selection elements established by Centene® clinical policy for elosulfase alfa (Vimizim®).

### Policy/Criteria

It is the policy of health plans affiliated with Centene Corporation® that Vimizim is **medically necessary** when the following criteria are met:

#### I. Initial Approval Criteria

##### A. Morquio A Syndrome (Mucopolysaccharidosis [MPS IVA]) (must meet all):

1. Diagnosis of Morquio A syndrome (MPS IVA) confirmed by one of the following:
  - a. Enzyme assay demonstrating a deficiency of N-acetylgalactosamine-6-sulfatase activity;
  - b. DNA testing.

**Approval duration: 6 months**

##### B. Other diagnoses/indications: Refer to CP.PHAR.57 - Global Biopharm Policy.

#### II. Continued Approval

##### A. Morquio A Syndrome (MPS IVA) (must meet all):

1. Currently receiving medication via Centene benefit or member has previously met all initial approval criteria;
2. Member is responding positively to therapy.

**Approval duration: 12 months**

##### B. Other diagnoses/indications (must meet 1 or 2):

1. Currently receiving medication via Centene benefit and documentation supports positive response to therapy; or
2. Refer to CP.PHAR.57 - Global Biopharm Policy.

### Background

#### *Description/Mechanism of Action:*

Mucopolysaccharidoses comprise a group of lysosomal storage disorders caused by the deficiency of specific lysosomal enzymes required for the catabolism of glycosaminoglycans (GAG). MPS IVA, also known as Morquio A syndrome, is characterized by the absence or marked reduction in N-acetylgalactosamine-6-sulfatase activity. The sulfatase activity deficiency results in the accumulation of the GAG substrates, KS and C6S, in the lysosomal compartment

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of cells throughout the body. The accumulation leads to widespread cellular, tissue, and organ dysfunction. Vimizim is intended to provide the exogenous enzyme N-acetylgalactosamine-6-sulfatase that will be taken up into the lysosomes and increase the catabolism of the GAGs KS and C6S. Elosulfase alfa uptake by cells into lysosomes is mediated by the binding of mannose-6-phosphate-terminated oligosaccharide chains of elosulfase alfa to mannose-6-phosphate receptors. In the absence of an animal disease model that recapitulates the human disease phenotype, elosulfase alfa pharmacological activity was evaluated using human primary chondrocytes from two MPS IVA patients. Treatment of MPS IVA chondrocytes with elosulfase alfa induced clearance of KS lysosomal storage from the chondrocytes.

*Formulations:*

Vimizim (elosulfase alfa): Solution for reconstitution; for intravenous use

- 5 mg/5 mL vial; 1 mg/mL (2.6 to 6.0 units/mg)

*FDA Approved Indications:*

Vimizim is a hydrolytic lysosomal glycosaminoglycan (GAG)-specific enzyme/intravenous formulation indicated for:

- Patients with Mucopolysaccharidosis type IVA (MPS IVA; Morquio A syndrome),

**Appendices**

**Appendix A: Abbreviation Key**

GAG: Glycosaminoglycan

MPS IVA: Mucopolysaccharidosis IVA

**Coding Implications**

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J1322	Injection, elosulfase alfa, 1 mg

Reviews, Revisions, and Approvals	Date	Approval Date
Policy split from CP.PHAR.48 Policy converted to new template	01/16	02/16
Age restriction removed. Allergy history is removed as the drug can be continued in some cases. Positive response to therapy added. Background section converted to new template. PI remains the same.	12/16	02/17

**References**

1. Vimizim prescribing information. Novato, CA: BioMarin Pharmaceutical, Inc.; February 2014. Available at <http://www.vimizim.com>. Accessed December 21, 2016.
2. Muenzer J. The mucopolysaccharidoses: a heterogeneous group of disorders with variable pediatric presentations. *J Pediatr.* 2004; 144(5 Suppl): S27-S34.

**Important Reminder**

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information. The Health Plan makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. “Health Plan” means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan’s affiliates, as applicable.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. The Health Plan retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment or medical care. It is not intended to dictate to providers how to practice medicine. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

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**Note: For Medicaid members,** when state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence. Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.

**Note: For Medicare members,** to ensure consistency with the Medicare National Coverage Determinations (NCD) and Local Coverage Determinations (LCD), all applicable NCDs and LCDs should be reviewed prior to applying the criteria set forth in this clinical policy. Refer to the CMS website at <http://www.cms.gov> for additional information.

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