



<b>CLINICAL MEDICAL POLICY</b>	
<b>Policy Name:</b>	VIMIZIM® (Elosulfase Alfa)
Policy Number:	MP-028-MD-PA
Approved By:	Medical Management
Provider Notice Date:	11/1/2016
Original Effective Date:	12/1/2016
Annual Approval Date:	7/19/2017
Revision Date:	NA
Products:	Pennsylvania Medicaid
Application:	All participating hospitals and providers
Page Number(s):	1 of 4

**DISCLAIMER:**

***Gateway Health<sup>SM</sup>'s (Gateway) medical payment and prior-authorization policy is intended to serve only as a general reference resource regarding payment and coverage for the services described. This policy does not constitute medical advice and is not intended to govern or otherwise influence medical decisions.***

<http://gatewayhealthplan.com/MedicalPolicies>

**POLICY STATEMENT:**

Gateway Health<sup>SM</sup> provides coverage for the intravenous administration of VIMIZIM® (elosulfase alfa) under the medical benefits for the Company's Medicaid products when medically necessary.

This policy is designed to address medical necessity guidelines that are appropriate for the majority of individuals with a particular disease, illness or condition. Each person's unique clinical circumstances warrants individual consideration, based upon review of applicable medical records.

(Current applicable Pennsylvania HealthChoices Agreement Section V. Program Requirements, B. Prior Authorization of Services, 1. General Prior Authorization Requirements.)

## **DEFINITIONS:**

**Prior Authorization Review Panel** – A panel of representatives from within the Pennsylvania Department of Human Services who have been assigned organizational responsibility for the review, approval and denial of all PH-MCO Prior Authorization policies and procedures.

**Mucopolysaccharidosis IV Type A** (i.e., MPS IVA, Morquio A syndrome) – a rare disorder in which the gene that encodes for N-acetylgalactosamine-6-sulfatase (GALNS) enzyme is mutated. This results in suboptimal GALNS activity that causes progressive accumulation of glycosaminoglycans in multiple organs and various tissues, leading to significant morbidities and multisystemic clinical impairments, resulting in diminished functional capacity, impaired quality of life, and early mortality.

## **PROCEDURES:**

1. Coverage may be provided for the administration of VIMIZIM® when the following medical necessity criteria are met:
  - a. The patient has been diagnosed with Mucopolysaccharidosis Type IV A (Morquio Syndrome); AND
  - b. The patient is age 5 years or older but is not over 65 years of age; AND
  - c. The diagnosis has been confirmed by a specialist conducting a biochemical evaluation, a peripheral blood leukocyte enzyme analysis, and a radiographic skeletal survey OR by performing DNA testing; AND
  - d. The prescriber is a biochemical geneticist or metabolic physician; AND
  - e. The product will be prepared and administered under the supervision of a health care professional able to manage medical emergencies in the event of an anaphylactic reaction; AND
  - f. The patient has some ability to walk at least 30 meters prior to treatment initiation. Documentation of a baseline six-minute walking test must be provided; AND
  - g. There is documentation of the patient's weight on a monthly basis; AND
  - h. The dosing is within the following prescribing-supported parameters: Dose does not exceed 2 mg/kg once a week; AND
  - i. Coverage will be continued when there is documentation of clinical improvement in walking and tolerance to the medication
2. When the VIMIZIM® services are not covered  
All other indications not otherwise listed are considered experimental/investigational unless peer reviewed medical literature or guidelines are provided to support its use.

When criteria are not met, the request will be forwarded to a Medical Director for review. The physician reviewer must override criteria when, in their professional judgment, the requested medication is medically necessary.

3. Post-payment Audit Statement  
The medical record must include documentation that reflects the medical necessity criteria and is subject to audit by Gateway Health<sup>SM</sup> at any time pursuant to the terms of your provider agreement.
4. Place of Service  
The place of service for the intravenous administration of VIMIZIM® is outpatient.

5. Governing Bodies Approval  
FDA

In 2014, The U.S. Food and Drug Administration (FDA) approved VIMIZIM® (elosulfase alfa), as the first FDA-approved treatment for Morquio A syndrome. VIMIZIM®, marketed by BioMarin Pharmaceutical Inc., is an enzyme replacement therapy for the missing GALNS enzyme.

Warnings:

- a. Anaphylaxis and Hypersensitivity Reactions: Life-threatening anaphylaxis and hypersensitivity reactions have been observed in some individuals during treatment with elosulfase alfa. If anaphylaxis or severe hypersensitivity reactions occur, immediately stop the infusion and initiate appropriate medical treatment. Pre-treatment with antihistamines with or without antipyretics is recommended prior to the start of infusion.
- b. Risk of Acute Respiratory Complications: Persons with acute febrile or respiratory illness may be at higher risk of life-threatening complications from hypersensitivity reactions. Careful consideration should be given to the patient's clinical status prior to administration of elosulfase alfa, and consider delaying the elosulfase infusion.
- c. Pregnancy Category C: There is a Morquio A Registry that collects data on pregnant women with MPS IVA who are treated with VIMIZIM®. Contact MARS@bmrn.com or call 1-800-983-4587 for information and enrollment.

There was no National Coverage Determination (NCD) located at the time of this medical policy development.

**CODING REQUIREMENTS:**

Procedure Codes

HCPC Codes	Description
J1322	Injection, elosulfase alfa, 1 mg

Diagnosis Codes

ICD-10 Diagnosis Codes	Description
E76.210	Morquio A mucopolysaccharidoses
E76.219	Morquio mucopolysaccharidoses, unspecified

**REIMBURSEMENT:**

Participating facilities will be reimbursed per their Gateway Health<sup>SM</sup> contract.

## **POLICY SOURCE(S):**

### Reference Sources

VIMIZIM® (elosulfase alpha) [package insert]. Novato, CA: BioMarin Pharmaceutical Inc.; 02/2014. Available at: <http://druginserts.com/lib/rx/meds/vimizim/>. Accessed on June 22, 2016.

Wynn, Robert. Mucopolysaccharidosis: Clinical features and diagnosis. *UpToDate*. Updated March, 2014. Accessed June 2016.

National Institute for Health and Care Excellence. Press and medial. NICE recommends elosulfase alfa (Vimizim) for treatment of very rare life-limiting genetic disorder under managed access agreement. 16 December 2015. Available at: <https://www.nice.org.uk/news/press-and-media/nice-recommends-elosulfase-alfa-vimizim-for-treatment-of-very-rare-life-limiting-genetic-disorder-under-managed-access-agreement>. Accessed on June 22, 2016.

National Organization for Rare Disorders (NORD). Morquio Syndrome. 2015. Available at: <http://rarediseases.org/rare-diseases/morquio-syndrome/>. Accessed June 22, 2016.