CLINICAL MEDICATION POLICY	
Policy Name:	VIMIZIM® (elosulfase alfa)
Policy Number:	MP-010-MC-ALL
Responsible Departments:	Medical Management, Medical Policy, Clinical Pharmacy
Provider Notice Date:	04/1/2017
Original Effective Date:	05/01/2017
Annual Approval Date:	03/15/2018
Revision Date:	N/A
Products:	Pennsylvania Medicare Assured
Application:	All participating and nonparticipating hospitals and providers
Page Number(s):	1 of 4

Disclaimer

Gateway Health℠ (Gateway) clinical medication 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.

POLICY STATEMENT

Gateway Health℠ may provide coverage under the medical or pharmacy benefits of the Company's Medicare products for medically necessary intravenous administration of Vimizim (elosulfase alfa).

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 warrant 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

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 aged 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 is not covered
   Vimizim is not covered for conditions other than those listed above because the scientific evidence has not been established.

   Coverage may be provided for any non-FDA labeled indication if it is determined that the use is a medically accepted indication supported by nationally recognized pharmacy compendia or peer-reviewed medical literature for treatment of the diagnosis(es) for which it is prescribed. These requests will be reviewed on a case-by-case basis to determine medical necessity.

   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. Warning
   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.

4. Post-payment Audit Statement
   The medical record must include documentation that reflects the medical necessity criteria and is subject to audit by Gateway Health℠ at any time pursuant to the terms of your provider agreement.

5. Place of Service
   The place of service for the intravenous administration of Vimizim is outpatient.

6. Coverage Determination
   Gateway Health℠ follows the coverage determinations made by CMS as outlined in either the national coverage determinations (NCD) or the state-specific local carrier determination (LCD).

   For Pennsylvania, there are no specific LCDs or NCDs for Vimizim. For additional information, please see:
   http://www.novitas-solutions.com/webcenter/portal/MedicalPolicy_JL/?_afrLoop=237596175793895#!%40%40%3F_afrLoop%3D237596175793895%26_adf.ctrl-state%3Dwnoa115p2_42

Governing Bodies Approval
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.

CODING REQUIREMENTS

Procedure Codes

CPT/HCPCS Codes	Description
J1322	Injection, elosulfase alfa, 1 mg

Diagnosis Codes

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

REMBURSEMENT

Participating facilities will be reimbursed per their Gateway Health℠ contract.

POLICY SOURCE(S)

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

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. Accessed on June 22, 2016 and 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.

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

Policy History:

Date	Activity
N/A	LCD/NCD effective date
03/15/2017	QI/UM Committee approval
05/01/2017	Provider effective date