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# POLICY Document for Vimizim

The overall objective of this policy is to support the appropriate and cost effective use of the medication, specific to use of lower cost site of care and overall clinically appropriate use. This document provides specific information to each section of the overall policy.

**Section 1: Site of Care**
- Policy information specific to site of care (outpatient, hospital outpatient, home infusion)

**Section 2: Clinical Criteria**
- Policy information specific to the clinical appropriateness for the medication

## Section 1: Site of Care

**Site of Care Criteria**
**Administration of Vimizim**

**POLICY**

**I. CRITERIA FOR APPROVAL FOR ADMINISTRATION IN OUTPATIENT HOSPITAL SETTING**

This policy provides coverage for administration of Vimizim in an outpatient hospital setting for up to 4 doses when a member is new to therapy.

This policy provides coverage for administration of Vimizim in an outpatient hospital setting for a longer course of treatment when ANY of the following criteria are met:

A. The member has experienced an adverse reaction that did not respond to conventional interventions (eg, acetaminophen, steroids, diphenhydramine, fluids or other pre-medications) or a severe adverse event (anaphylaxis, anaphylactoid reactions, myocardial infarction, thromboembolism, or seizures) during or immediately after an infusion.
B. The member is medically unstable (eg respiratory, cardiovascular, or renal conditions).
C. The member has severe venous access issues that require the use of a special intervention.
D. The member has significant behavioral issues and/or physical or cognitive impairment that would impact the safety of the infusion therapy AND the patient does not have access to a caregiver
E. Alternative infusion sites are not available.
F. The member is less than 21 years of age or 65 years of age or older.

For situations where administration of Vimizim does not meet the criteria for outpatient hospital infusion, coverage for Vimizim is provided when administered in alternative sites such as; physician office, home infusion or ambulatory care.

**II. REQUIRED DOCUMENTATION**

The following information is necessary to initiate the site of care prior authorization review (where applicable):

A. Medical records supporting the member has experienced an adverse reaction that did not respond to conventional interventions or a severe adverse event during or immediately after an infusion
B. Medical records supporting the member is medically unstable.
C. Medical records supporting the member has severe venous access issues.

D. Medical records supporting the member has behavioral issues and/or physical or cognitive impairment and no access to a caregiver
E. Records supporting alternative infusion sites are not available

# Section 2: Clinical Criteria

## SPECIALTY GUIDELINE 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 Indications
Vimizim is indicated for patients with Mucopolysaccharidosis IVA (MPS IVA, Morquio A syndrome).

All other indications are considered experimental/investigational and are not a covered benefit.

**II. CRITERIA FOR INITIAL APPROVAL**

**Mucopolysaccharidosis IVA (MPS IVA)**
Indefinite authorization 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.

**III. CONTINUATION OF THERAPY**

All members (including new members) requesting authorization for continuation of therapy must meet all initial authorization criteria.

## REFERENCES:


### SECTION 1

1. Vimizim [package insert]. Novato, CA: BioMarin Pharmaceutical Inc; February 2014.
2. Long B, Tompkins T, Decker C, et al. Long-term Immunogenicity of Elosulfase Alfa in the Treatment of Morquio A Syndrome: Results From MOR-005, a Phase III Extension Study. *Clin Ther.* 2017;39(1):118-129 e113.
3. Hendriksz CJ, Burton B, Fleming TR, et al. Efficacy and safety of enzyme replacement therapy with BMN 110 (elosulfase alfa) for Morquio A syndrome (mucopolysaccharidosis IVA): a phase 3 randomized placebo-controlled study. *J Inherit Metab Dis.* 2014;37(6):979-990.
4. Hendriksz CJ, Parini R, AlSayed MD, et al. Long-term endurance and safety of elosulfase alfa enzyme replacement therapy in patients with Morquio A syndrome. *Mol Genet Metab.* 2016;119(1-2):131-143.
5. Harmatz PR, Mengel E, Geberhiwot T, et al. Impact of elosulfase alfa in patients with morquio A syndrome who have limited ambulation: An open-label, phase 2 study. *Am J Med Genet A.* 2017;173(2):375-383.

### SECTION 2

1. Vimizim [package insert]. Novato, CA: BioMarin Pharmaceutical Inc.; February 2014.