Clinical Policy: Elosulfase Alfa (Vimizim)

Reference Number: PA.CP.PHAR.162
Effective Date: 01/18
Last Review Date: 04/19

Coding Implications
Revision Log

Description
Elosulfase alfa (Vimizim®) is a hydrolytic lysosomal glycosaminoglycan-specific enzyme.

FDA Approved Indication
Vimizim is indicated for patients with mucopolysaccharidosis type IVA (MPS IVA; Morquio A syndrome).

Policy/Criteria
It is the policy of Pennsylvania Health and Wellness that Vimizim is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Morquio A Syndrome (Mucopoloysaccharidosis [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.
2. Age ≥ 5 years;
3. Dose does not exceed 2 mg/kg/week.

Approval duration: 6 months

B. Other diagnoses/indications: Refer to PA.CP.PMN.53

II. Continued Approval

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

1. Currently receiving medication via Pennsylvania Health and Wellness benefit or member has previously met all initial approval criteria; or the Continuity of Care policy (PA.LTSS.PHAR.01) applies;
2. Member is responding positively to therapy as evidenced by improvement in the individual member's MPS IVA disease manifestation profile *(see Appendix D for examples)*;
3. If request is for a dose increase, new dose does not exceed 2 mg/kg/week.

Approval duration: 12 months

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

1. Currently receiving medication via Pennsylvania Health and Wellness benefit and documentation supports positive response to therapy; or the Continuity of Care policy (PA.LTSS.PHAR.01) applies; or
2. Refer to PA.CP.PMN.53

III. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

FDA: Food and Drug Administration
MPS IVA: mucopolysaccharidosis IVA

Appendix B: Therapeutic Alternatives
Not applicable

Appendix C: Contraindications/Boxed Warnings
- Contraindication(s): none reported.
- Boxed warning(s): risk of life-threatening anaphylactic reactions during Vimizim infusions.

Appendix D: General Information
The presenting symptoms and clinical course of MPS IVA can vary from one individual to another. Some examples, however, of improvement in MPS IVA disease as a result of Vimizim therapy may include improvement in:
- 6-minute walking test distance
- Breathing difficulties
- Muscle weakness
- Vision or hearing problems
- Hepatomegaly or splenomegaly

IV. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
MPS IVA	2 mg/kg IV once weekly	2 mg/kg/week

V. Product Availability
Single-use vial: 5 mg/5 mL

VI. References
1. Vimizim Prescribing Information. Novato, CA: BioMarin Pharmaceutical, Inc.; February 2014. Available at http://www.vimizim.com. Accessed February 28, 2019.
2. Muenzer J. The mucopolysaccharidoses: a heterogeneous group of disorders with variable pediatric presentations. J Pediatr. 2004; 144(5 Suppl): S27-S34.
3. Hendriksz CJ, Berger KI, Giugliani R, et al. International guidelines for the management and treatment of Morquio A syndrome. Am J Med Genet A. 2015; 167(1): 11-25.

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
2Q 2018 annual review: age restriction added; references reviewed and updated.	2.27.18	
2Q 2019 annual review: references reviewed and updated.	04/19	