Clinical Policy: Elosulfase Alfa (Vimizim)

Reference Number: PA.CP.PHAR.162
Effective Date: 01/2018
Last Review Date: 04/2025

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
Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

It is the policy of PA Health & 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 or b):
   a. Enzyme assay demonstrating a deficiency of N-acetylgalactosamine-6-sulfatase activity;
   b. DNA testing.
2. Age ≥ 5 years;
3. Documentation of member's current weight (in kg);
4. 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 PA Health & Wellness benefit or member has previously met all initial approval criteria; or the Continuity of Care policy (PA.PHARM.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. Documentation of member's current weight (in kg);
4. 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 PA Health & Wellness benefit and documentation supports positive response to therapy; or the Continuity of Care policy (PA.PHARM.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
- Height and weight
- 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.; December 2019. Available at http://www.vimizim.com. Accessed January 8, 2025.
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.

4. Akyol MU, Alden TD, Amartino H, et al. Recommendations for the management of MPS IVA: systematic evidence- and consensus-based guidance. Orphanet J of Rare Dis 2019;14(137):1-25.
5. Stapleton M, Hoshina H, Sawamoto K, et al. Critical review of current MPS guidelines and management. Molecular Genetics and Metabolism. 2019;126:238-45.
6. Sawamoto K, Alvarez Gonzalez JV, Piechnik M, et al. Mucopolysaccharidosis IVA: diagnosis, treatment, and management. Intl J Molecular Sciences. 2020;21:1517; doi:10.3390/ijms21041517.

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
2Q 2018 annual review: age restriction added; references reviewed and updated.	02/2018
2Q 2019 annual review: references reviewed and updated.	04/2019
2Q 2020 annual review: references reviewed and updated.	04/2020
2Q 2021 annual review: references reviewed and updated.	04/2021
2Q 2022 annual review: added requirement for documentation of current weight for dose calculation purposes; references reviewed and updated.	04/2022
2Q 2023 annual review: no significant changes; references reviewed and updated.	04/2023
2Q 2024 annual review: no significant changes; references reviewed and updated.	04/2024
2Q 2025 annual review: no significant changes; references reviewed and updated.	04/2025