# Clinical Policy: Elosulfase Alfa (Vimizim)

Reference Number: PA.CP.PHAR.162
Effective Date: 01/2018
Last Review Date: 04/2026

**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: 12 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 including any of the following: 6-minute walking test distance, breathing difficulties, muscle weakness, vision or hearing problems, height and weight, hepatomegaly, or splenomegaly;
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): hypersensitivity reactions including anaphylaxis and risk of acute respiratory complications

*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.; October 2025. Available at http://www.vimizim.com. Accessed January 14, 2026.
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 |
| 2Q 2026 annual review: no significant changes; updated initial approval duration from 6 months to 12 months; for Continued Therapy added examples of positive treatment response that were already outlined in Appendix D and previously referred to within the criteria; references reviewed and updated. | 04/2026 |