

## Vimizim (elosulfase alfa) (Intravenous)

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### I. Length of Authorization

Coverage will be provided for 12 months and may be renewed.

### II. Dosing Limits

Max Units (per dose and over time) [HCPSC Unit]:

- 920 billable units every 28 days

### III. Initial Approval Criteria <sup>1,3</sup>

*Submission of supporting clinical documentation (including but not limited to medical records, chart notes, lab results, and confirmatory diagnostics) related to the medical necessity criteria is REQUIRED on all requests for authorizations. Records will be reviewed at the time of submission as part of the evaluation of this request. Please provide documentation related to diagnosis, step therapy, and clinical markers (i.e., genetic, and mutational testing) supporting initiation when applicable. Please provide documentation via direct upload through the PA web portal or by fax. Failure to submit the medical records may result in the denial of the request due to inability to establish medical necessity in accordance with policy guidelines.*

Coverage is provided in the following conditions:

- Patient is at least 5 years of age; **AND**
- Documented baseline for one or more of the following:
  - Endurance tests (e.g., six-minute walk test [6-MWT], timed 25-foot walk test [T25FW], three-minute stair climb test [3-MSCT], etc.)
  - Pulmonary function tests (e.g., maximum voluntary ventilation [MVV], percent predicted forced vital capacity [FVC], etc.)
  - Urine keratan sulfate (KS) or urine glycosaminoglycan (GAG) levels

#### **Mucopolysaccharidosis Type IVA (MPS IVA; Morquio A Syndrome) † ☐ <sup>1,3-6</sup>**

- Documented diagnosis of Mucopolysaccharidosis type IVA with biochemical/genetic confirmation by one of the following:
  - Absence or marked reduction in N-acetylgalactosamine 6-sulfatase (GALNS) enzyme activity in cultured fibroblasts or leukocytes; **OR**

- Detection of biallelic pathogenic mutations in the *GALNS* gene by genetic molecular testing (i.e., sequence analysis and/or deletion/duplication analysis)

† FDA Approved Indication(s); ‡ Compendia Recommended Indication(s); Ⓢ Orphan Drug

#### IV. Renewal Criteria <sup>1,3,5</sup>

Coverage may be renewed based on the following criteria:

- Patient continues to meet the indication-specific relevant criteria such as concomitant therapy requirements (not including prerequisite therapy), performance status, etc. identified in section III; **AND**
- Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include: anaphylaxis and hypersensitivity reactions, acute respiratory complications, spinal/cervical cord compression, etc.; **AND**
- Patient has shown a response to therapy as evidenced by one or more of the following when compared to pretreatment baseline:
  - Stability or improvement in endurance tests (e.g., six-minute walk test [6-MWT], timed 25-foot walk test [T25FW], three-minute stair climb test [3-MSCT], etc.)
  - Stability or improvement in pulmonary function tests (e.g., maximum voluntary ventilation [MVV], percent predicted forced vital capacity [FVC], etc.)
  - Stability or reduction in urine keratan sulfate (KS) or urine glycosaminoglycan (GAG) levels

#### V. Dosage/Administration <sup>1</sup>

| Indication  | Dose   |
|---|--|
| Mucopolysaccharidosis IVA (MPS IVA; Morquio A Syndrome) | Administer 2 mg/kg intravenously once every week |

#### VI. Billing Code/Availability Information

HCPCS Code:

- J1322 – Injection, elosulfase alfa, 1 mg; 1 billable unit = 1 mg

NDC:

- Vimizim 5 mg/5 mL solution in a single-dose vial: 68135-0100-xx

#### VII. References

1. Vimizim [package insert]. Novato, CA; BioMarin Pharmaceutical Inc.; December 2019. Accessed March 2025.
2. 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 Jan; 167(1): 11–25. Published online 2014 Oct 24. Doi: 10.1002/ajmg.a.36833

3. Regier DS, Oetgen M, Tanpaiboon P. Mucopolysaccharidosis Type IVA. 2013 Jul 11 [Updated 2021 Jun 17]. In: Adam MP, Feldman J, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2025. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK148668/>. Accessed March 2025.
4. Schweighardt B, Tompkins T, Lau K, et al. Immunogenicity of Elosulfase Alfa, an Enzyme Replacement Therapy in Patients With Morquio A Syndrome: Results From MOR-004, a Phase III Trial. Clin Ther. 2015 May 1;37(5):1012-1021.e6. doi: 10.1016/j.clinthera.2014.11.005. Epub 2014 Dec 6.
5. 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 randomised placebo-controlled study. J Inherit Metab Dis. 2014 Nov;37(6):979-90. Doi: 10.1007/s10545-014-9715-6. Epub 2014 May 9.
6. Zhou J, Lin J, Leung WT, Wang L. A basic understanding of mucopolysaccharidosis: Incidence, clinical features, diagnosis, and management. Intractable Rare Dis Res. 2020 Feb;9(1):1-9. Doi: 10.5582/irdr.2020.01011.
7. Shapiro EG, Eisengart JB. The natural history of neurocognition in MPS disorders: A review. Mol Genet Metab. 2021 May;133(1):8-34. Doi: 10.1016/j.ymgme.2021.03.002. Epub 2021 Mar 11. PMID: 33741271.

## Appendix 1 – Covered Diagnosis Codes

| ICD-10  | Description                     |
|---------|---------------------------------|
| E76.210 | Morquio A mucopolysaccharidoses |

## Appendix 2 – Centers for Medicare and Medicaid Services (CMS)

The preceding information is intended for non-Medicare coverage determinations. Medicare coverage for outpatient (Part B) drugs is outlined in the Medicare Benefit Policy Manual (Pub. 100-2), Chapter 15, §50 Drugs and Biologicals. In addition, National Coverage Determinations (NCDs) and/or Local Coverage Determinations (LCDs) may exist and compliance with these policies is required where applicable. Local Coverage Articles (LCAs) may also exist for claims payment purposes or to clarify benefit eligibility under Part B for drugs which may be self-administered. The following link may be used to search for NCD, LCD, or LCA documents: <https://www.cms.gov/medicare-coverage-database/search.aspx>. Additional indications, including any preceding information, may be applied at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCD/LCA): N/A

| Medicare Part B Administrative Contractor (MAC) Jurisdictions |  |   |
|---|--|---|
| Jurisdiction  | Applicable State/US Territory          | Contractor  |
| E (1)   | CA, HI, NV, AS, GU, CNMI               | Noridian Healthcare Solutions, LLC                |
| F (2 & 3)   | AK, WA, OR, ID, ND, SD, MT, WY, UT, AZ | Noridian Healthcare Solutions, LLC                |
| 5   | KS, NE, IA, MO                         | Wisconsin Physicians Service Insurance Corp (WPS) |
| 6   | MN, WI, IL                             | National Government Services, Inc. (NGS)          |

| Medicare Part B Administrative Contractor (MAC) Jurisdictions |   |   |
|---|---|---|
| Jurisdiction  | Applicable State/US Territory   | Contractor  |
| H (4 & 7)   | LA, AR, MS, TX, OK, CO, NM  | Novitas Solutions, Inc.                           |
| 8   | MI, IN  | Wisconsin Physicians Service Insurance Corp (WPS) |
| N (9)   | FL, PR, VI  | First Coast Service Options, Inc.                 |
| J (10)  | TN, GA, AL  | Palmetto GBA                                      |
| M (11)  | NC, SC, WV, VA (excluding below)  | Palmetto GBA                                      |
| L (12)  | DE, MD, PA, NJ, DC (includes Arlington & Fairfax counties and the city of Alexandria in VA) | Novitas Solutions, Inc.                           |
| K (13 & 14)   | NY, CT, MA, RI, VT, ME, NH  | National Government Services, Inc. (NGS)          |
| 15  | KY, OH  | CGS Administrators, LLC                           |