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

A. Quantity Limit (max daily dose) [NDC Unit]:

- Vimizim 5 mg/5 mL single-dose vial: 184 vials every 28 days

B. Max Units (per dose and over time) [HCPCS Unit]:

- 230 billable units (230 mg) every 7 days

III. Initial Approval Criteria [1,3]

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])
  - 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) † Φ [1,3-6]

- 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); Φ Orphan Drug

IV. Renewal Criteria [1,3.5]

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])
  - 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 [1]

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 January 2024.

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
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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)
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, LLC
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA, LLC
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