Vimizim® (elosulfase alfa)
(Intravenous)

Effective Date: 01/01/2020
Review Date: 12/20/2019, 11/23/2020, 7/15/2021, 02/17/2022, 01/26/2023, 12/07/2023, 01/04/2024, 01/15/2025, 02/17/2026

Scope: Medicaid, Commercial, Medicare

I. Length of Authorization

Coverage will be for 6 months and may be renewed.

II. Dosing Limits

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

- 230 billable units (230mg) every 7 days

III. Summary of Evidence

Vimizim (elosulfase alfa) is a hydrolytic lysosomal glycosaminoglycan (GAG)-specific enzyme indicated for the treatment of Morquio A syndrome (mucopolysaccharidosis type IVA), a rare lysosomal storage disorder. Clinical trials evaluating its efficacy and safety have demonstrated significant improvements in endurance, respiratory function, and walking capacity in patients with Morquio A syndrome. In phase 3 studies, Vimizim has shown to improve the 6-minute walk test distance and respiratory function, as evidenced by improvements in forced vital capacity (FVC) and maximum voluntary ventilation (MVV). These improvements in respiratory function contribute to enhanced quality of life and reduced respiratory complications in patients with Morquio A syndrome. Adverse events commonly reported include infusion-related reactions, fever, and headache.

IV. Initial Approval Criteria[1,4,5,6]

Coverage is provided in the following conditions:

Medicare members who have previously received this medication within the past 365 days are not subject to Step Therapy Requirements.

Universal Criteria

- Member is 5 years of age or older; **AND**

Mucopolysaccharidosis Type IVA (MPS IVA, Morquio A Syndrome)

- 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); **AND**
- Documented baseline value for one or more of the following:
  - Endurance tests [e.g., six minute walk test (6-MWT) or 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.

V. Renewal Criteria[1,4,5,6]

- Member continues to meet universal and other indication-specific relevant criteria such as concomitant therapy requirements (not including prerequisite therapy), performance status, etc. identified in section IV; **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**
- Member has shown a response to therapy as evidenced byone or more of the following markers when compared to pretreatment baseline values:
  - 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., FVC, etc.)
  - Stability or reduction in urine keratan sulfate (KS) or urine glycosaminoglycan (GAG)

VI. Dosage/Administration[1,4,5,6]

Indication	Dose
Mucopolysaccharidosis Type IVA (MPS IVA; Morquio A Syndrome)	2 mg/kg administered once every week as an intravenous (IV) infusion over 3.5 to 4.5 hours. - Infusion rate o Initial: ▪ Less than 25 kg – 3 mL/hour*

	▪ Greater than 25 kg – 6 mL/hour* o Maximum: ▪ Less than 25 kg – 36 mL/hour ▪ Greater than 25 kg – 72 mL/hour o *If tolerated, the infusion rate may be increased incrementally by 6 mL/hour for patients < 25 kg and 12 mL/hour for patients ≥ 25 kg to maximum infusion rate. - Minimum infusion time: o Less than 25 kg – 3.5 hours o Greater than 25 kg – 4.5 hours

VII. Billing Code/Availability Information

HCPCS Code:

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

NDC:

- Vimizim 5mg/5ml injection: 68135-0100-xx

VIII. References

1. Vimizim [package insert]. Novato, CA; Biomarin Pharmaceutical Inc.; October 2025. Accessed January 2065.
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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 Jan; 167(1): 11–25. Published online 2014 Oct 24. doi: 10.1002/ajmg.a.36833
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5. 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.
6. 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.

7. 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.
8. 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)

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/Articles): 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)

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	**Applicable State/US Territory**	**Contractor**
15	KY, OH	CGS Administrators, LLC

Policy Rationale:

Vimizim was reviewed by the Neighborhood Health Plan of Rhode Island Pharmacy & Therapeutics (P&T) Committee. Neighborhood adopted the following clinical coverage criteria to ensure that its members use Vimizim according to Food and Drug Administration (FDA) approved labeling and/or relevant clinical literature. Neighborhood worked with network prescribers and pharmacists to draft these criteria. These criteria will help ensure its members are using this drug for a medically accepted indication, while minimizing the risk for adverse effects and ensuring more cost-effective options are used first, if applicable and appropriate. For Medicare members, these coverage criteria will only apply in the absence of National Coverage Determination (NCD) or Local Coverage Determination (LCD) criteria. Neighborhood will give individual consideration to each request it reviews based on the information submitted by the prescriber and other information available to the plan.