

Vimizim® (elosulfase alfa) (Intravenous)

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I. Length of Authorization

Coverage will be for 12 months and may be renewed.

II. Dosing Limits

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

- Vimizim 5mg/5ml: 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^{1,4,5,6}

Coverage is provided in the following conditions:

Universal Criteria

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

Mucopolysaccharidosis IVA (MPS IVA, Morquio A Syndrome)

- Documented diagnosis of Mucopolysaccharidosis IVA with biochemical/genetic confirmation by one of the following:
 - Absence or marked reduction in N-acetylgalactosamine 6-sulfatase (GALNS) enzyme activity; **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)), and/or pulmonary function tests (e.g., FVC), etc.

IV. Renewal Criteria^{1,4,5,6}

- Patient 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 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 markers when compared to pretreatment baseline values:
 - Stability or improvement in six minute walk test (6-MWT), three minute stair climb test (3-MSCT)
 - Stability or improvement in pulmonary function tests

V. Dosage/Administration^{1,4,5,6}

Indication	Dose
Mucopolysaccharidosis IVA	2 mg/kg administered once every week as an intravenous (IV) infusion

VI. Billing Code/Availability Information

HCPCS Code:

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

NDC:

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

VII. References

1. Vimizim [package insert]. Novato, CA; Biomarin Pharmaceutical Inc.; December 2019. Accessed January 2020.
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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
4. Regier DS, Oetgen M, Tanpaiboon P. Mucopolysaccharidosis Type IVA. 2013 Jul 11 [Updated 2016 Mar 24]. In: Adam MP, Ardinger HH, Pagon RA, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2019. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK148668/>

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 Inher Metab Dis. 2014 Nov;37(6):979-90. doi: 10.1007/s10545-014-9715-6. Epub 2014 May 9.

Appendix 1 – Covered Diagnosis Codes

ICD-10	Description
E76.210	Morquio A mucopolysaccharidoses

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

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 Determination (NCD), Local Coverage Determinations (LCDs), and Articles may exist and compliance with these policies is required where applicable. They can be found at: <http://www.cms.gov/medicare-coverage-database/search/advanced-search.aspx>. Additional indications may be covered 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)
15	KY, OH	CGS Administrators, LLC