

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 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

Coverage is provided in the following conditions:

- Patient is at least 5 years of age; **AND**
- Documented baseline value 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]), and/or pulmonary function tests (e.g., percent predicted forced vital capacity [FVC]), etc.; **AND**

Mucopolysaccharidosis IVA (MPS IVA; Morquio A Syndrome) † ☐ ^{1,3-5}

- 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 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 ^{1,3,5}

Coverage may be renewed based on the following criteria:

- Patient continues to meet 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 the following 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]); **OR**
 - Stability or improvement in pulmonary function tests (e.g., FVC, etc.)

V. Dosage/Administration ¹

Indication	Dose
Mucopolysaccharidosis IVA (MPS IVA; Morquio A Syndrome)	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 5 mg/5 ml solution; single-dose vial: 68135-0100-xx

VII. References

1. Vimizim [package insert]. Novato, CA; Biomarin Pharmaceutical Inc.; December 2019. Accessed January 2023.
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. GeneReviews® <https://www.ncbi.nlm.nih.gov/books/NBK148668/>. Initial Posting: July 11, 2002; Last Update: June 17, 2021. Accessed on January 6, 2023.
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.

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 Local Coverage Articles (LCAs) may exist and compliance with these policies is required where applicable. They can be found at: <https://www.cms.gov/medicare-coverage-database/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/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

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Provides free aids and services to people with disabilities to communicate effectively with us, such as:

- Qualified sign language interpreters
- Written information in other formats (large print, audio, accessible electronic formats, other formats)

Provides free language services to people whose primary language is not English, such as:

- Qualified interpreters
- Information written in other languages

If you need these services, contact a Grievance Specialist.

If you believe that PCHP has failed to provide these services or discriminated in another way on the basis of race, color, national origin, age, disability, or sex, you can file a grievance with:

Grievance Specialist
PreferredOne Community Health Plan
PO Box 59052
Minneapolis, MN 55459-0052
Phone: 1.800.940.5049 (TTY: 763.847.4013)
Fax: 763.847.4010
customerservice@preferredone.com

You can file a grievance in person or by mail, fax, or email. If you need help filing a grievance, a Grievance Specialist is available to help you.

You can also file a civil rights complaint with the U.S. Department of Health and Human Services, Office for Civil Rights, electronically through the Office for Civil Rights Complaint Portal, available at <https://ocrportal.hhs.gov/ocr/portal/lobby.jsf>, or by mail or phone at:

U.S. Department of Health and Human Services
200 Independence Avenue, SW
Room 509F, HHH Building
Washington, D.C. 20201
1-800-368-1019, 800-537-7697 (TDD)

Complaint forms are available at <http://www.hhs.gov/ocr/office/file/index.html>.

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ATTENTION: If you do not speak English, language assistance services, free of charge, are available to you. Call 1.800.940.5049 (TTY: 763.847.4013).

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LUS CEEV: Yog tias koj hais lus Hmoob, cov kev pab txog lus, muaj kev pab dawb rau koj. Hu rau 1.800.940.5049 (TTY: 763.847.4013).

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Minneapolis, MN 55459-0212
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200 Independence Avenue, SW
Room 509F, HHH Building
Washington, D.C. 20201
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