Vimizim (elosulfase alfa)
(Intravenous)

Document Number: MH-0190

Last Review Date: 02/01/2022
Date of Origin: 04/29/2014
Dates Reviewed: 02/2015, 01/2016, 01/2017, 01/2018, 02/2019, 02/2020, 02/2021, 02/2022

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 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]

Coverage is provided in the following conditions:

Patient is required to meet Site of Service specialty infusion program requirements (refer to the Medica Site of Service Policy).

Submission of medical records (chart notes) related to the medical necessity criteria is REQUIRED on all requests for authorizations. Records will be reviewed at the time of submission. Please provide documentation related to diagnosis, step therapy, and clinical markers (i.e. genetic and mutational testing) supporting initiation when applicable. Medical records may be submitted via direct upload through the PA web portal or by fax.

- 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) † Φ [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); Φ Orphan Drug

IV. Renewal Criteria [1,3.5]

- 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 5mg per5ml solution; single-dose vial: 68135-0100-xx

VII. References

1. Vimizim [package insert]. Novato, CA; Biomarin Pharmaceutical Inc.; December 2019. Accessed January 2022.
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 21, 2022.
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