Elosulfase Alfa (Vimizim®)

NDC CODE(S) 68135-0100-XX VIMIZIM 5MG/5ML Solution (BIOMARIN PHARMACEUTICALS)

DESCRIPTION

Elosulfase alfa is a purified human enzyme produced by recombinant DNA technology. It is used for enzyme replacement therapy for individuals with mucopolysaccharidosis IVA (MPS IVA, Morquio A syndrome). Mucopolysaccharidoses are a group of lysosomal storage disorders caused by the deficiency of specific enzymes required for the catabolism of glycosaminoglycans (GAG). The enzyme deficient or absent in MPS IVA is N-acetylgalactosamine-6-sufatase leading to accumulation of the GAG substrates keratan sulfate (KS) and chondroitin-6-sulfate (C6S) throughout the body, eventually leading to widespread cellular, tissue and organ dysfunction.

POLICY

- Elosulfase alfa for the treatment of mucopolysaccharidosis IVA is considered medically necessary if the medical appropriateness criteria are met. (See Medical Appropriateness below.)
- Elosulfase alfa for the treatment of other conditions/diseases is considered investigational.

MEDICAL APPROPRIATENESS

INITIAL APPROVAL CRITERIA

- Patient is at least 5 years of age**; 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.

RENEWAL CRITERIA

- Patient continues to meet indication-specific relevant criteria such as concomitant therapy requirements (not including prerequisite therapy), performance status, etc. identified in initial approval criteria; **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 endurance test (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

DOSAGE/ADMINISTRATION

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

LENGTH OF AUTHORIZATION

Coverage will be for 12 months and may be renewed.

DOSAGE LIMITS

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

- 230 billable units (230 mg) every 7 days

APPLICABLE TENNESSEE STATE MANDATE REQUIREMENTS

BlueCross BlueShield of Tennessee's Medical Policy complies with Tennessee Code Annotated Section 56-7-2352 regarding coverage of off-label indications of Food and Drug Administration (FDA) approved drugs when the off-label use is recognized in one of the statutorily recognized standard reference compendia or in the published peer-reviewed medical literature.

IMPORTANT REMINDER

We develop Medical Policies to provide guidance to Members and Providers. This Medical Policy relates only to the services or supplies described in it. The existence of a Medical Policy is not an authorization, certification, explanation of benefits or a contract for the service (or supply) that is referenced in the Medical Policy. For a determination of the benefits that a Member is entitled to receive under his or her health plan, the Member's health plan must be reviewed. If there is a conflict between the Medical Policy and a health plan, the express terms of the health plan will govern.

ADDITIONAL INFORMATION

For appropriate chemotherapy regimens, dosage information, contraindications, precautions, warnings, and monitoring information, please refer to one of the standard reference compendia (e.g., the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) published by the National Comprehensive Cancer Network®, Drugdex Evaluations of Micromedex Solutions at Truven Health, or The American Hospital Formulary Service Drug Information).

SOURCES

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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 Inherit Metab Dis. 2014 Nov;37(6):979-90. doi: 10.1007/s10545-014-9715-6. Epub 2014 May 9.
7. Lexi-Comp Online. (2020, March). AHFS DI. Elosulfase alfa. Retrieved February 1, 2021 from Lexi-Comp Online with AHFS.
8. MICROMEDEX Healthcare Series. Drugdex Evaluations. (2020, January). Elosulfase alfa. Retrieved February 1, 2021 from MICROMEDEX Healthcare Series.

EFFECTIVE DATE 6/30/2021

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