Bright HealthCare™

Medical Policy

Vimizim (elosulfase alfa)	
MEDICAL POLICY NUMBER	Med_Clin_Ops-061
CURRENT VERSION EFFECTIVE DATE	January 1, 2024
APPLICABLE PRODUCT AND MARKET	*Individual Family Plan:* All Plans *Small Group*: All Plans *Medicare Advantage:* All Plans

Brand New Day/Central Health Medicare Plan develops policies and makes coverage determinations using credible scientific evidence including but not limited to MCG™ Health Guidelines, the ASAM Criteria™, and other third party sources, such as peer-reviewed medical literature generally recognized by the relevant medical community, physician specialty society recommendations, and expert opinion as relevant to supplement those sources. Brand New Day/Central Health Medicare Plan Medical Policies, MCG™ Guidelines, and the ASAM Criteria™ are not intended to be used without the independent clinical judgment of a qualified health care provider considering the individual circumstances of each member's case. The treating health care providers are solely responsible for diagnosis, treatment, and medical advice. Members may contact Brand New Day/Central Health Medicare Plan Customer Service at the phone number listed on their member identification card to discuss their benefits more specifically. Providers with questions about this Brand New Day/Central Health Medicare Plan Medical Policy may contact the Health Plan. Brand New Day/Central Health Medicare Plan policies and practices are compliant with federal and state requirements, including mental health parity laws.

If there is a difference between this policy and the member specific plan document, the member benefit plan document will govern. For Medicare Advantage members, Medicare National Coverage Determinations (NCD) and Local Coverage Determinations (LCD), govern. Refer to the CMS website at http://www.cms.gov for additional information.

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PURPOSE

To promote consistency between reviewers in clinical coverage decision-making by providing the criteria that generally determine the medical necessity of Vimizim (elosulfase alfa) therapy.

POLICY/CRITERIA

Prior Authorization and Medical Review is required.

Coverage for Vimizim will be provided for 12 months and may be renewed.

Initial Therapy

1. Patient has a diagnosis of Mucopolysaccharidosis Type IVA (Morquio A Syndrome) confirmed by **one** of the following:
    a. Absence or deficiency of fibroblast or leukocyte Galactosamine (N-Acetyl)-6-Sulfatase (GALNS) enzyme activity; **OR**
    b. Molecular genetic testing for mutations in the GALNS gene; **AND**

2. Patient is displaying signs and symptoms of the disease e.g., kyphoscoliosis, genu valgum, pectus carinatum, gait disturbance, growth deficiency, etc.); **AND**
3. Vimizim is prescribed by or in consultation with a geneticist, endocrinologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of lysosomal storage disorders.

Continuation Therapy

1. Patient has experienced a positive clinical response to elosulfase alfa therapy (e.g., improved endurance, improved functional capacity, reduced urine keratan sulfate excretion).

LIMITATIONS/EXCLUSIONS

1. Any indication other than those listed above due to insufficient evidence of therapeutic value

BACKGROUND

Mucopolysaccharidoses comprise a group of lysosomal storage disorders caused by the deficiency of specific lysosomal enzymes required for the catabolism of glycosaminoglycans (GAG). Mucopolysaccharidosis IVA (MPS IVA, Morquio A Syndrome) is characterized by the absence or marked reduction in N-acetylgalactosamine-6-sulfatase activity. The sulfatase activity deficiency results in the accumulation of the GAG substrates, KS and C6S, in the lysosomal compartment of cells throughout the body. The accumulation leads to widespread cellular, tissue, and organ dysfunction. Vimizim (elosulfase alfa) is intended to provide the exogenous enzyme N-acetylgalactosamine-6-sulfatase that will be taken up into the lysosomes and increase the catabolism of the GAGs KS and C6S. Elosulfase alfa uptake by cells into lysosomes is mediated by the binding of mannose-6-phosphate-terminated oligosaccharide chains of elosulfase alfa to mannose-6-phosphate receptors.

DEFINITIONS

1. VIMIZIM (elosulfase alfa) injection, for intravenous use. Initial U.S. Approval: 2014
    a. Vimizim is supplied as a concentrated solution for infusion (1 mg per mL) requiring dilution. One vial of 5 mL contains 5 mg Vimizim.

CODING

Applicable NDC Codes	
68135-0100-xx	Vimizim 1mg/1ml, 5ml vial injection

Applicable Procedure Code	
J1322	Vimizim 5 mg injection; 1 billable unit = 1 mg

Applicable ICD-10 Codes	
E76.210	Morquio A mucopolysaccharidoses

EVIDENCE BASED REFERENCES

1. Vimizim® injection for intravenous use [prescribing information]. Novato, CA: BioMarin Pharmaceuticals; December 2019.
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;167A:11-25.
3. Tomatsu S, Yasuda E, Patel P, et al. Morquio A syndrome: Diagnosis and current and future therapies. *Pediatr Endocrinol Rev*. 2014;12:141-151.
4. Regier DS, Tanpaiboon P. Role of elosulfase alfa in mucopolysaccharidosis IVA. *Appl Clin Genet*. 2016;9:67-74.

POLICY HISTORY

Original Effective Date	May 24, 2021
Revised Date	November 1, 2021: Annual review – no changes made. February 22, 2022: Annual review – no changes made. February 28, 2023 – Annual Review and approval (no policy revisions made) March 1, 2023 – Adopted by MA UM Committee (no policy revisions made)

Approved by Pharmacy and Therapeutics 2/28/23