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Elosulfase Alfa

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INSTRUCTIONS FOR USE
The following Coverage Policy applies to health benefit plans administered by Cigna Companies. Certain Cigna Companies and/or lines of business only provide utilization review services to clients and do not make coverage determinations. References to standard benefit plan language and coverage determinations do not apply to those clients. Coverage Policies are intended to provide guidance in interpreting certain standard benefit plans administered by Cigna Companies. Please note, the terms of a customer's particular benefit plan document [Group Service Agreement, Evidence of Coverage, Certificate of Coverage, Summary Plan Description (SPD) or similar plan document] may differ significantly from the standard benefit plans upon which these Coverage Policies are based. For example, a customer's benefit plan document may contain a specific exclusion related to a topic addressed in a Coverage Policy. In the event of a conflict, a customer's benefit plan document always supersedes the information in the Coverage Policies. In the absence of a controlling federal or state coverage mandate, benefits are ultimately determined by the terms of the applicable benefit plan document. Coverage determinations in each specific instance require consideration of 1) the terms of the applicable benefit plan document in effect on the date of service; 2) any applicable laws/regulations; 3) any relevant collateral source materials including Coverage Policies and; 4) the specific facts of the particular situation. Coverage Policies relate exclusively to the administration of health benefit plans. Coverage Policies are not recommendations for treatment and should never be used as treatment guidelines. In certain markets, delegated vendor guidelines may be used to support medical necessity and other coverage determinations.

Overview

This policy supports medical necessity review for elosulfase alfa intravenous infusion (**Vimizim**®).

Receipt of sample product does not satisfy any criteria requirements for coverage.

Medical Necessity Criteria

Elosulfase alfa (Vimizim) is considered medically necessary when the following are met:

1. **Mucopolysaccharidosis Type IVA (MPS IVA, Morquio A Syndrome).** Individual meets **BOTH** of the following criteria (A and B):
    A. The diagnosis is established by one of the following (i or ii):
        i. Individual has a laboratory test demonstrating deficient N-acetylgalactosamine 6-sulfatase (*GALNS*) activity in leukocytes or fibroblasts

ii. Individual has a molecular genetic test demonstrating biallelic pathogenic or likely pathogenic N-acetylgalactosamine 6-sulfatase (*GALNS*) gene variants

B. The medication 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.

When coverage is available and medically necessary, the dosage, frequency, duration of therapy, and site of care should be reasonable, clinically appropriate, and supported by evidence-based literature and adjusted based upon severity, alternative available treatments, and previous response to therapy.

Reauthorization Criteria

Elosulfase alfa (Vimizim) is considered medically necessary for continued use when initial criteria are met AND there is documentation of beneficial response.

Authorization Duration

Initial and reauthorization approval duration: 12 months

Conditions Not Covered

Any other use is considered experimental, investigational or unproven.

Coding / Billing Information

Note:

1) This list of codes may not be all-inclusive.
2) Deleted codes and codes which are not effective at the time the service is rendered may not be eligible for reimbursement.

Considered Medically Necessary when criteria in the applicable policy statements listed above are met:

HCPCS Codes	Description
J1322	Injection, elosulfase alfa, 1 mg

Background

OVERVIEW

Vimizim, a human *N*-acetylgalactosamine-6-sulfatase, is indicated for patients with **Mucopolysaccharidosis type IVA** (Morquio A syndrome [MPS IVA]).[1] It is produced in Chinese hamster ovary cells via recombinant DNA technology. Vimizim is a hydrolytic lysosomal enzyme which is taken up by lysosomes and hydrolyzes sulfate from the non-reduced ends of the glycosaminoglycans keratan sulfate and chondroitin-6-sulfate.

Disease Overview

MPS IVA (Morquio A syndrome) is a rare lysosomal storage disorder characterized by deficient *N*-acetylgalactosamine-6-sulfatase activity leading to the accumulation of chondroitin-6-sulfate and keratan sulfate in lysosomes in bone, cartilage, and ligaments.[2,3] The clinical course, onset, and severity of MPS IVA is heterogeneous.[2] Manifestations of MPS IVA include short trunk dwarfism with short neck, kyphoscoliosis, odontoid dysplasia, knock-knee, cervical spinal cord compression, hypermobile joints, cardiac disease, respiratory insufficiency, obstructive sleep apnea, corneal clouding, and dental abnormalities.[2-4] MPS IVA has not been associated with cognitive decline.[2] The definitive diagnosis of MPS IVA is established by demonstrating deficient *N*-acetylgalactosamine-6-sulfatase activity in leukocytes or fibroblasts; or by genetic

testing.[2] Definitive treatment for MPS IVA consists of enzyme replacement therapy with Vimizim. Hematopoietic stem cell transplantation is not recommended for MPS IVA.

Dosing and Availability
The recommended dosage is 2 mg per kg of body weight administered once weekly as an intravenous infusion. Elosulfase alfa is supplied as a concentrated solution for infusion (1 mg per mL) requiring dilution. One vial of 5 mL contains 5 mg elosulfase alfa.

References

1. Vimizim® intravenous infusion [prescribing information]. Novato, CA: BioMarin; January 2021.
2. Akyol MU, et al. MPS Consensus Programme Co-Chairs. Recommendations for the management of MPS IVA: systematic evidence- and consensus-based guidance. *Orphanet J Rare Dis*. 2019 Jun 13;14(1):137.
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.