Clinical Policy: Elosulfase Alfa (Vimizim)

Reference Number: PA.CP.PHAR.162
Effective Date: 01/18
Last Review Date: 04/18

Coding Implications
Revision Log

Description
The intent of the criteria is to ensure that patients follow selection elements established by Pennsylvania Health and Wellness® clinical policy for elosulfase alfa (Vimizim®).

FDA Approved Indication
Vimizim is indicated for patients with mucopolysaccharidosis type IVA (MPS IVA; Morquio A syndrome).

Policy/Criteria
It is the policy of Pennsylvania Health and Wellness that Vimizim is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Morquio A Syndrome (Mucopoloysaccharidosis [MPS IVA]) (must meet all):

1. Diagnosis of Morquio A syndrome (MPS IVA) confirmed by one of the following:
   a. Enzyme assay demonstrating a deficiency of N-acetylgalactosamine-6-sulfatase activity;
   b. DNA testing.
2. Age ≥ 5 years;
3. Dose does not exceed 2 mg/kg/week.

Approval duration: 6 months

B. Other diagnoses/indications: Refer to PA.CP.PHAR.57 - Global Biopharm Policy.

II. Continued Approval

A. Morquio A Syndrome (MPS IVA) (must meet all)**:**

1. Currently receiving medication via Pennsylvania Health and Wellness benefit or member has previously met all initial approval criteria; or the Continuity of Care policy (PA.LTSS.PHAR.01) applies;
2. Member is responding positively to therapy as evidenced by improvement in the individual member's MPS IVA disease manifestation profile *(see Appendix C for examples)*;
3. If request is for a dose increase, new dose does not exceed 2 mg/kg/week.

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2)**:**

1. Currently receiving medication via Pennsylvania Health and Wellness benefit and documentation supports positive response to therapy; or the Continuity of Care policy (PA.LTSS.PHAR.01) applies; or
2. Refer to PA.CP.PHAR.57 - Global Biopharm Policy**.**

Background

Description/Mechanism of Action:

Mucopolysaccharidoses comprise a group of lysosomal storage disorders caused by the deficiency of specific lysosomal enzymes required for the catabolism of glycosaminoglycans (GAG). MPS IVA, also known as 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 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. In the absence of an animal disease model that recapitulates the human disease phenotype, elosulfase alfa pharmacological activity was evaluated using human primary chondrocytes from two MPS IVA patients. Treatment of MPS IVA chondrocytes with elosulfase alfa induced clearance of KS lysosomal storage from the chondrocytes.

Formulations:

Vimizim (elosulfase alfa): Solution for reconstitution; for intravenous use

- 5 mg/5 mL vial; 1 mg/mL (2.6 to 6.0 units/mg)

Appendices

Appendix A: Abbreviation Key

GAG: GlycosaminoglycanMPS IVA: Mucopoloysaccharidosis IVA

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: General Information

The presenting symptoms and clinical course of MPS IVA can vary from one individual to another. Some examples, however, of improvement in MPS IVA disease as a result of Vimizim therapy may include improvement in:

- 6-minute walking test distance
- Breathing difficulties
- Muscle weakness
- Vision or hearing problems
- Hepatomegaly or splenomegaly

Coding Implications

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J1322	Injection, elosulfase alfa, 1 mg

Reviews, Revisions, and Approvals	Date	Approval Date
2Q 2018 annual review: age restriction added; references reviewed and updated.	2.27.18	

References

1. Vimizim prescribing information. Novato, CA: BioMarin Pharmaceutical, Inc.; February 2014. Available at http://www.vimizim.com. Accessed February 27, 2018.
2. Muenzer J. The mucopolysaccharidoses: a heterogeneous group of disorders with variable pediatric presentations. *J Pediatr*. 2004; 144(5 Suppl): S27-S34.