Vimizim (elosulfase alfa)

Override(s)	Approval Duration
Prior Authorization	1 year

Medications	Dosing Limit
Vimizim (elosulfase alfa) 5 mg vial	2 mg/kg once per week

APPROVAL CRITERIA

Initial requests for Vimizim (elosulfase alfa) may be approved if the following criteria are met:

I. Individual has a diagnosis of mucopolysaccharidosis IVA (Morquio A syndrome); **AND**
II. Documentation is provided that diagnosis is demonstrated by (Akyol 2019; Wood 2013):
    A. Reduced fibroblast or leukocyte N-acetylgalactosamine-6-sulfatase (GALNS) enzyme activity combined with normal enzyme activity level of another sulfatase; **OR**
    B. *GALNS* genetic mutation;

AND

III. Individual demonstrates clinical signs and symptoms of Morquio A syndrome (for example, knee deformity, corneal opacity or pectus carinatum) (Hendriksz 2015; Wood 2013)**.**

Continuation requests for Vimizim (elosulfase alfa) may be approved if the following criterion is met:

I. Documentation is provided to show clinically significant improvement or stabilization in clinical signs and symptoms of disease (including but not limited to reduction in urinary GAG excretion, reduction in hepatosplenomegaly, improvement in pulmonary function, improvement in walking distance and/or improvement in fine or gross motor function) compared to the predicted natural history trajectory of disease.

Requests for Vimizim (elosulfase alfa) may not be approved for the following:

I. All other indications not included above; **OR**
II. Individual is using to treat mucopolysaccharidosis IVB (Morquio B syndrome).

Note:
Vimizim (elosulfase alfa) has a black box warning for anaphylaxis. Life-threatening anaphylactic reactions have occurred during Vimizim infusions so appropriate medical support should be available during Vimizim administration. Individuals should be educated on the signs and symptoms of anaphylaxis and to seek immediate medical care should they occur.

Individuals with acute respiratory illness may be at risk of serious acute exacerbation of their respiratory disease and require additional monitoring.

Key References:

1. Akyol MU, Alden TD, Amartino H, et al. Recommendations for the management of MPS IVA: systematic evidence and consensus-based guidance. *Orphanet J Rare Dis*. 2019;14(1):137. doi: 10.1186/s13023-019-1074-9
2. DailyMed. Package inserts. U.S. National Library of Medicine, National Institutes of Health website. http://dailymed.nlm.nih.gov/dailymed/about.cfm. Accessed: September 9, 2023.
3. DrugPoints® System [electronic version]. Truven Health Analytics, Greenwood Village, CO. Updated periodically.
4. Hendriksz CJ, Berger KI, Giugliani R, at al. International guidelines for the management and treatment of Morquio A syndrome. *Am J Med Genet A*. 2015; 167A(1):11-25.
5. Lehman TJ, Miller N, Norquist B, Underhill L, Keutzer J. Diagnosis of the mucopolysaccharidoses. *Rheumatology (Oxford)*. 2011;50 Suppl 5:v41-v48. doi:10.1093/rheumatology/ker390.
6. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc. Updated periodically.
7. Wood TC, Harvey K, Beck M, et al. Diagnosing mucopolysaccharidosis IVA. *J Inherit Metab Dis*. 2013;36:293–307.

Federal and state laws or requirements, contract language, and Plan utilization management programs or polices may take precedence over the application of this clinical criteria.