

Vimizim® (elosulfase alfa)

When requesting Vimizim® (elosulfase alfa), the individual requiring treatment must be diagnosed with the following FDA-approved indication and meet the specific coverage guidelines and applicable safety criteria for the covered indication.

FDA-approved Indication

Vimizim (elosulfase alfa) is indicated for the treatment of individuals with mucopolysaccharidosis type IVA (MPS IVA; Morquio A syndrome).

Coverage Guidelines

Mucopolysaccharidosis Type IVA (MPS IVA; Morquio A Syndrome)

The individual must meet all of the following criteria for approval:

- Diagnosis is confirmed by one of the following:
 - o Laboratory test demonstrating deficient N-acetylgalactosamine-6-sulfatase activity in leukocytes or fibroblasts; OR
 - o Molecular genetic test demonstrating biallelic pathogenic or likely pathogenic N-acetylgalactosamine-6-sulfatase gene variants;
- Vimizim is prescribed by or in consultation with a geneticist, an endocrinologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of lysosomal storage disorders.

Approval duration: 12 months

Dosing Recommendation

The recommended dose is 2 mg/kg given intravenously over a minimum range of 3.5 to 4.5 hours, based on infusion volume, once every week.

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*.