Vimizim® (elosulfase alfa)

October 2020

Background:
Mucopolysaccharidosis (MPS) IVA or Morquio A syndrome is an autosomal recessive lysosomal storage disorder (LSD) caused by deficiency of the N-acetylgalactosamine-6-sulfatase (GALNS) enzyme, which impairs lysosomal degradation of keratan sulphate and chondroitin-6-sulphate.

Vimizim is a hydrolytic lysosomal glycosaminoglycan (GAG)-specific enzyme indicated for patients with Mucopolysaccharidosis type IVA (MPS IVA; Morquio A syndrome)

Criteria for approval:

Initial approval duration: 6 months

1. Patient is 5 years of age or older; **AND**
2. Patient has a diagnosis of Mucopolysaccharidosis IV type A (MPS IVA, Morquio A syndrome); **AND**
3. Diagnosis has been confirmed by one of the following:
   a. Genetic testing; **OR**
   b. Absence or deficiency in N-acetylgalactosamine 6-sulfatase (GALNS) enzyme activity; **AND**
4. At least one of the following baseline testing has been completed and will be used to assess response to therapy:
   a. Endurance test [i.e., Distance walked in six minutes (6-MWT) or Timed 25-foot walk (T25FW)]; **OR**
   b. Pulmonary test [i.e., Forced vital capacity (FVC), Forced expiration volume in 1 second (FEV_1), or Maximal voluntary ventilation (MVV)]; **AND**
5. Documented clinical signs and symptoms of Morquio A syndrome (for example, kyphoscoliosis, pectus carinatum, knee deformity, etc.)
6. Patient does not have any contraindication(s) to the requested medication; **AND**
7. Medication is being prescribed by or in consultation with an endocrinologist, geneticist, metabolic disorders specialist, or an expert in the disease state; **AND**
8. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence; **AND**
9. Weight must be received for drugs that have weight-based dosing.
10. Vimizim will be administered under the supervision of a healthcare professional with the capability to manage anaphylaxis.

Continuation of therapy:

Renewal approval duration: 12 months

1. Patient has responded to treatment as demonstrated by an improvement and/or stabilization compared to baseline in at least one of the following:
    a. Endurance test [e.g., Distance walked in six minutes (6-MWT), Rate of stair climbing in three minutes (3-MSCT), or Timed 25-foot walk (T25-FW)]; **OR**
    b. Pulmonary test [e.g., Forced vital capacity (FVC), Forced expiration volume in 1 second (FEV_1), or Maximal voluntary ventilation (MVV)]; **AND**
2. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence; **AND**
3. For dose increase requests, weight must be received for drugs that have weight-based dosing.

Note: Vimizim has a black box warning:

- Life-threatening anaphylactic reactions have occurred in some patients during Vimizim infusions.
- Anaphylaxis, presenting as cough, erythema, throat tightness, urticaria, flushing, cyanosis, hypotension, rash, dyspnea, chest discomfort, and gastrointestinal symptoms in conjunction with urticaria, have been reported to occur during infusions, regardless of duration of the course of treatment.
- Closely observe patients during and after Vimizim administration and be prepared to manage anaphylaxis.

References:

1. Vimizim [Product information]. BioMarin Pharmaceutical Inc. Novato, CA; 12/2019.
2. Jones S, et al. Mucopolysaccharidoses: Clinical features and diagnosis. UpToDate. From: https://www.uptodate.com (Accessed on May 6, 2020.)
3. Genetics Home Reference. U.S. National Library of Medicine. Mucopolysaccharidosis type IV. From: https://ghr.nlm.nih.gov/condition/mucopolysaccharidosis-type-iv (Accessed on May 6, 2020)
4. Vimizim [Website]. BioMarin Pharmaceutical Inc. From: https://www.vimizim.com/about-vimizim/how-vimizim-can-help. (Accessed on May 6, 2020).
5. Akyol MU et al. Recommendations for the management of MPS IVA: systematic evidence- and consensus-based guidance. Orphanet Journal of Rare Diseases volume 14: 137. 2019. (Accessed on August 26, 2020)