BlueCross BlueShield of Arizona
An Independent Licensee of the Blue Cross and Blue Shield Association

MEDICAL COVERAGE GUIDELINES
SECTION: DRUGS

ORIGINAL EFFECTIVE DATE: 07/09/14
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VIMIZIM™ (elosulfase alfa)

Coverage for services, procedures, medical devices and drugs are dependent upon benefit eligibility as outlined in the member's specific benefit plan. This Medical Coverage Guideline must be read in its entirety to determine coverage eligibility, if any.

The section identified as "Description" defines or describes a service, procedure, medical device or drug and is in no way intended as a statement of medical necessity and/or coverage.

The section identified as "Criteria" defines criteria to determine whether a service, procedure, medical device or drug is considered medically necessary or experimental or investigational.

State or federal mandates, e.g., FEP program, may dictate that any drug, device or biological product approved by the U.S. Food and Drug Administration (FDA) may not be considered experimental or investigational and thus the drug, device or biological product may be assessed only on the basis of medical necessity.

Medical Coverage Guidelines are subject to change as new information becomes available.

For purposes of this Medical Coverage Guideline, the terms "experimental" and "investigational" are considered to be interchangeable.

Description:

Vimizim is a hydrolytic lysosomal glycosaminoglycan (GAG)-specific enzyme indicated for individuals with Mucopolysaccharidosis type IVA (also known as MPS IVA or Morquio A syndrome). Individuals born with MPS IV do not produce enough of the Nacetylgalactosamine-6 sulfatase (Type IVA) or beta-galactosidase (Type IVB) enzyme needed to break down large sugar molecules called glycosaminoglycans (GAGs). GAGs then build up in tissues, bones and major organs.

Clinical features are similar in both types but appear milder in MPS IVB. Onset is between ages 1 and 3. Neurological complications include spinal nerve and nerve root compression resulting from extreme, progressive skeletal changes, particularly in the ribs and chest; conductive and/or neurosensitive loss of hearing and clouded corneas. Intelligence is normal unless hydrocephalus develops and is not treated. Physical growth slows and often stops around age 8. Skeletal abnormalities include a bell-shaped chest, a flattening or curvature of the spine, shortened long bones, and dysplasia of the hips, knees, ankles, and wrists. The bones that stabilize the connection between the head and neck can be malformed (odontoid hypoplasia). Restricted breathing, joint stiffness and heart disease are also common.

MPS IVA is caused by mutations in the GALNS gene and MPS IVB is caused by mutations in the GLB1 gene.

VIMIZIM (elosulfase alfa) (cont.)

Criteria:

- FDA-approved dosage of Vimizim for the treatment of mucopolysaccharidosis type IVA in individuals 5 years of age and older is considered ***medically necessary*** for a maximum of 24 weeks of treatment.

- Vimizim for all other indications not previously listed or if above criteria not met is considered ***experimental or investigational*** based upon:

  1. Lack of final approval from the Food and Drug Administration, and
  2. Insufficient scientific evidence to permit conclusions concerning the effect on health outcomes, and
  3. Insufficient evidence to support improvement of the net health outcome, and
  4. Insufficient evidence to support improvement of the net health outcome as much as, or more than, established alternatives, and
  5. Insufficient evidence to support improvement outside the investigational setting.

Resources:

1. Vimizim™ Package Insert. Accessed 06/06/2014.

2. National Institute of Neurological Disorders and Stroke. Mucopolysaccharidoses Fact Sheet. Accessed 06/09/2014.

3. National Library of Medicine. Genetics Home Reference: Mucopolysaccharidosis type IV. Accessed 06/09/2014.

FDA Product Approval Information for Vimizim:

- FDA-approved indication: For patients with mucopolysaccharidosis type IVA (MPS IVA; Morquio A syndrome).

- FDA-approved dosage: 2 mg per kg given intravenously over a minimum range of 3.5 to 4.5 hours, based on infusion volume, once every week. Pre-treatment with antihistamines with or without antipyretics is recommended 30 to 60 minutes prior to the start of the infusion.