Galsulfase

DESCRIPTION

Galsulfase is a recombinant purified human enzyme. Within the body, the natural enzyme, *N*-acetylgalactosamine 4-sulfatase, is a lysosomal glycosaminoglycan (GAG)-specific enzyme required for the removal of the GAG substrate, dermatan sulfate, throughout the body. Without the natural enzyme, the accumulation of dermatan sulfate leads to widespread cellular, tissue and organ dysfunction.

Individuals with an absence or marked shortage of the endogenous enzyme are diagnosed with the mucopolysaccharide storage disorder of mucopolysaccharidosis VI (MPS VI or Maroteaux-Lamy syndrome). Clinical symptoms of MPS VI include skeletal deformities, organ and soft tissue involvement. Specific abnormalities may include short stature, abnormal bone formation, degenerative joint disease, hydrocephalus, impaired vision and hearing, sleep disorders, reduced endurance, coarse facial features, carpal tunnel syndrome, spastic quadriplegia, hepatomegaly, splenomegaly, cardiac valve dysfunction, respiratory dysfunction and shortened life span to between childhood and early adulthood.

The proposal is to add text/statements in red and to delete text/statements with strikethrough:

POLICY

- Galsulfase for the treatment of mucopolysaccharidosis VI (MPS VI, Maroteaux-Lamy syndrome) is considered ***medically necessary*** if the medical appropriateness criteria are met. **(See Medical Appropriateness below.)**
- Galsulfase for the treatment of other conditions/diseases is considered ***investigational.***

MEDICAL APPROPRIATENESS

INITIAL APPROVAL

- Galsulfase is considered **medically appropriate** if **ALL** of the following criteria are met:
  - Diagnosis of mucopolysaccharidosis VI (MPS VI, Maroteaux-Lamy syndrome) confirmed by **ANY ONE** of the following:
    - Detection of pathogenic mutations in the ARSB gene by molecular genetic testing
    - Arylsulfatase B (ASB) enzyme activity of <10% of the lower limit of normal in cultured fibroblasts or isolated leukocytes and **ALL** of the following:
      - Individual has normal enzyme activity of a different sulfatase (excluding patients with Multiple Sulfatase Deficiency [MSD])
      - Individual has an elevated urinary GAG level as defined as being above the upper limit of normal by the reference laboratory
    - ~~Detection of pathogenic mutations in the ARSB gene by molecular genetic testing~~
  - Individual is 5 years of age or older
  - Documented baseline 12-minute walk test (12-MWT),~~and/or a baseline~~ 3-minute stair climb test, and/or pulmonary function tests (e.g., FEV1, etc.)
  - Documented baseline value for urinary glycosaminoglycan (uGAG)

RENEWAL CRITERIA

- Galsulfase is considered **medically appropriate** for renewal if **ALL** of the following criteria are met:
  - Individual continues to meet the initial approval criteria
  - Disease response to treatment as defined by reduction in uGAG levels and improvement ~~in~~ or stability from pre-treatment baseline for **ANY ONE** of the following:
    - 12-minute walk test (12-MWT) ~~and/or~~

- 3-minute stair climb test compared to pre-treatment baseline
- Pulmonary function testing (e.g., FEV1, etc.)

- Absence of unacceptable toxicity from the agent, including, but not limited to, anaphylaxis and allergic reactions; immune mediated reactions; acute respiratory complications; acute cardiorespiratory reactions; severe infusion reactions, spinal or cervical cord compression, etc.

INDICATION(S)	DOSAGE & ADMINISTRATION
Mucopolysaccharidosis VI (Maroteaux-Lamy syndrome)	1mg/kg of body weight administered as an intravenous infusion over no less than 4 hours once a week

LENGTH OF AUTHORIZATION

Coverage will be provided for 12 months and may be renewed.

Refer to **DOSAGE LIMITS** below

APPLICABLE TENNESSEE STATE MANDATE REQUIREMENTS

BlueCross BlueShield of Tennessee's Medical Policy complies with Tennessee Code Annotated Section 56-7-2352 regarding coverage of off-label indications of Food and Drug Administration (FDA) approved drugs when the off-label use is recognized in one of the statutorily recognized standard reference compendia or in the published peer-reviewed medical literature.

IMPORTANT REMINDER

We develop Medical Policies to provide guidance to Members and Providers. This Medical Policy relates only to the services or supplies described in it. The existence of a Medical Policy is not an authorization, certification, explanation of benefits or a contract for the service (or supply) that is referenced in the Medical Policy. For a determination of the benefits that a Member is entitled to receive under his or her health plan, the Member's health plan must be reviewed. If there is a conflict between the Medical Policy and a health plan, the express terms of the health plan will govern.

ADDITIONAL INFORMATION

For appropriate chemotherapy regimens, dosage information, contraindications, precautions, warnings, and monitoring information, please refer to one of the standard reference compendia (e.g., the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) published by the National Comprehensive Cancer Network®, Drugdex Evaluations of Micromedex Solutions at Truven Health, or The American Hospital Formulary Service Drug Information).

SOURCES

Lexi-Comp Online. (2018). AHFS DI. *Galsulfase*. Retrieved November 28, 2018 from Lexi-Comp Online with AHFS.

MICROMEDEX Healthcare Series. Drugdex Drug Evaluations. (2017, August). *Galsulfase*. Retrieved November 28, 2018 from MICROMEDEX Healthcare Series.

U. S. Food and Drug Administration. (2013, March). Center for Drug Evaluation and Research. *Naglazyme® (galsulfase) injection for intravenous use.* Retrieved March 28, 2018 from http://www.accessdata.fda.gov/drugsatfda_docs/label/2013/125117s111lbl.pdf.

Vairo F, Federhen A, Baldo G, et al. Diagnostic and treatment strategies in mucopolysaccharidosis VI. Appl Clin Genet. 2015 Oct 30;8:245-55.

EFFECTIVE DATE

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