Approved Revision: Do Not Implement Until 6/2/21

Idursulfase (Elaprase®)

NDC CODE(S) 54092-0700-XX ELAPRASE 6MG/3ML Solution (SHIRE US INC)

DESCRIPTION

Idursulfase is a purified form of the lysosomal enzyme iduronate-2-sulfatase. It is produced by recombinant DNA technology in a human cell line. This enzyme is required for systemic elimination of the glycosaminoglycans (GAGs) dermatan sulfate and heparan sulfate. If the enzyme is missing or defective, GAGs progressively accumulate in the lysosomes of nearly all cell types, leading to cellular engorgement, organomegaly, tissue destruction and organ system dysfunction. This condition is known as Mucopolysaccharidosis II, MPS II or Hunter syndrome. A rare disease, it is the only known X-linked recessive mucopolysaccharidosis disorder.

MPS II is generally manifest in two forms: Severe disease, affecting two-thirds of those diagnosed, in which death typically occurs in the mid-teenage years due to chronic progressive disease of neurological deterioration and cardiorespiratory failure. The other third have attenuated disease with survival into adulthood, although death frequently occurs between the ages of 20 and 30 years from cardiac or respiratory disease.

While not curative, enzyme replacement therapy with idursulfase can improve quality of life if administered early in the disease state. Additional supportive treatment is generally required for symptomatic control.

POLICY

- Idursulfase for the treatment of Mucopolysaccharidosis II (i.e., MPS II, Hunter syndrome) is considered ***medically necessary*** if the medical appropriateness criteria are met. **(See Medical Appropriateness below.)**
- Idursulfase for the treatment of other conditions/diseases is considered ***investigational.***

MEDICAL APPROPRIATENESS

INITIAL APPROVAL CRITERIA

- Patient is at least 16 months of age; **AND**
- Patient has absence of severe cognitive impairment; **AND**
  - Documented baseline value for urinary glycosaminoglycan (uGAG) has been obtained; **AND**
  - Documented baseline values for one or more of the following have been obtained:
    - Patients 5 years or greater: 6-minute walk test (6-MWT), percent predicted forced vital capacity (FVC), joint range of motion, left ventricular hypertrophy, growth, quality of life (CHAQ/HAQ/MPS HAQ); **OR**
    - Patients < 5 years: spleen volume, liver volume, FVC, and/or 6-minute walk test; **AND**

Hunter syndrome (Mucopolysaccharidosis II; MPS II)

- Diagnosis has been confirmed by one of the following:
  - Deficient or absent iduronate 2-sulfatase (I2S) enzyme activity in white cells, fibroblasts, or plasma in the presence of normal activity of at least one other sulfatase; **OR**
  - Detection of pathogenic mutations in the IDS gene by molecular genetic testing

RENEWAL CRITERIA

- Patient continues to meet universal and other indication-specific relevant criteria identified in the Initial Approval Criteria; **AND**

- Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include: severe hypersensitivity reactions including anaphylaxis, antibody development and serious adverse reactions in Hunter Syndrome patients with severe genetic mutations, acute respiratory complications, acute cardiorespiratory failure, etc.; **AND**
- Patient does not have progressive/irreversible severe cognitive impairment; **AND**
- Patient has a documented reduction in uGAG levels; **AND**
- Patient has demonstrated a beneficial response to therapy compared to pretreatment baseline in one or more of the following:
  - Patients 5 years or greater: stabilization or improvement in percent predicted FVC and/or 6-minute walk test, increased joint range of motion, decreased left ventricular hypertrophy, improved growth, improved quality of life (clinically meaningful change in the CHAQ/HAQ/MPS HAQ disability index); **OR**
  - Patients < 5 years: reductions in spleen volume, and/or liver volume or stabilization/improvement in FVC and/or 6-MWT

DOSAGE/ADMINISTRATION

INDICATION	DOSE
Hunter Syndrome; MPS II	0.5 mg/kg of body weight administered once weekly as an intravenous infusion

LENGTH OF AUTHORIZATION

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

DOSAGE LIMITS

Max Units (per dose and over time) [HCPCS Unit]:

- 60 billable units every 7 days

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

1. Elaprase [package insert]. Lexington, MA; Shire Human Genetic Therapies, Inc; November 2018. Accessed December 2020.
2. Wraith JE, Scarpa M, Beck M, et al. Mucopolysaccharidosis type II (Hunter syndrome): a clinical review and recommendations for treatment in the era of enzyme replacement therapy. Eur J Pediatr. 2008 ar;167(3):267-77. Epub 2007 Nov 23.
3. Scarpa M, Almássy Z, Beck M, et al. Mucopolysaccharidosis type II: European recommendations for the diagnosis and multidisciplinary management of a rare disease. Orphanet J Rare Dis. 2011 Nov 7;6:72. doi: 10.1186/1750-1172-6-72.
4. Muenzer J, Bodamer O, Burton B, et al. The role of enzyme replacement therapy in severe Hunter syndrome-an expert panel consensus. Eur J Pediatr. 2012 Jan;171(1):181-8.
5. Scarpa M. Mucopolysaccharidosis Type II. GeneReviews®. www.ncbi.nlm.nih.gov/books/NBK1274/. Initial Posting: November 6, 2007; Last Update: October 4, 2018. Accessed on December 26, 2019.
6. Burrow T, Leslie ND. Review of the use of idursulfase in the treatment of mucopolysaccharidosis II. Biologics. 2008 Jun; 2(2): 311–320.
7. Giugliani R, Villareal MLS, Valdez CAA, et al. Guidelines for diagnosis and treatment of Hunter Syndrome for clinicians in Latin America. Genet Mol Biol. 2014 Jun; 37(2): 315–329.
8. Burton BK, Giugliani R. Diagnosing Hunter syndrome in pediatric practice: practical considerations and common pitfalls. Eur J Pediatr 2012; 171:631.
9. Muenzer J, Wraith J, Beck M, et al. A phase II/III clinical study of enzyme replacement therapy with idursulfase in mucopolysaccharidosis II (Hunter syndrome). Genet Med 8, 465– 473 (2006) doi:10.1097/01.gim.0000232477.37660.fb
10. Muenzer J, Beck M, Eng CM, et al. Long-term, open-labeled extension study of idursulfase in the treatment of Hunter syndrome. Genet Med. 2011 Feb;13(2):95-101. doi: 10.1097/GIM.0b013e3181fea459.
11. Lexicomp Online. (2021). AHFS DI. Idursulfase. Retrieved February 5, 2021 from Lexicomp Online with AHFS.
12. MICROMEDEX Healthcare Series. Drugdex Drug Evaluations. (2019, November). Idursulfase. Retrieved February 5, 2021 from MICROMEDEX Healthcare Series.

EFFECTIVE DATE 6/2/2021

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