Reference number(s)
4810-A

STANDARD MEDICARE PART B MANAGEMENT

ELAPRASE (idursulfase)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications
Elaprase is indicated for patients with Hunter syndrome (Mucopolysaccharidosis II, MPS II). Elaprase has been shown to improve walking capacity in patients 5 years and older. In patients 16 months to 5 years of age, no data are available to demonstrate improvement in disease-related symptoms or long term clinical outcome; however, treatment with Elaprase has reduced spleen volume similarly to that of adults and children 5 years of age and older. The safety and efficacy of Elaprase have not been established in pediatric patients less than 16 months of age.

All other indications will be assessed on an individual basis. Submissions for indications other than those enumerated in this policy should be accompanied by supporting evidence from Medicare approved compendia.

II. DOCUMENTATION

The following documentation must be available, upon request, for all submissions:
A. Initial requests: iduronate 2-sulfatase enzyme assay or genetic testing results supporting diagnosis.
B. Continuation requests: chart notes documenting a clinically positive response to therapy, which shall include improvement, stabilization, or slowing of disease progression.

III. CRITERIA FOR INITIAL APPROVAL

Mucopolysaccharidosis II (MPS II)
Authorization of 12 months may be granted for treatment of MPS II when the diagnosis of MPS II was confirmed by enzyme assay demonstrating a deficiency of iduronate 2-sulfatase enzyme activity or by genetic testing.

IV. CONTINUATION OF THERAPY

All members (including new members) requesting authorization for continuation of therapy must be currently receiving therapy with the requested agent.

Authorization for 12 months may be granted when all of the following criteria are met:
A. The member is currently receiving therapy with Elaprase
B. Elaprase is being used to treat an indication enumerated in Section III

C. The member is receiving benefit from therapy. Benefit is defined as a clinically positive response to therapy, which shall include improvement, stabilization, or slowing of disease progression.

V. SUMMARY OF EVIDENCE

The contents of this policy were created after examining the following resources:

1. The prescribing information for Elaprase.
2. The available compendium
    a. National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium
    b. Micromedex DrugDex
    c. American Hospital Formulary Service- Drug Information (AHFS-DI)
    d. Lexi-Drugs
    e. Clinical Pharmacology
3. Recognition and diagnosis of mucopolysaccharidosis II
4. Multidisciplinary management of Hunter syndrome

After reviewing the information in the above resources, the FDA-approved indications listed in the prescribing information for Elaprase are covered.

VI. EXPLANATION OF RATIONALE

Support for FDA-approved indications can be found in the manufacturer's prescribing information.

Support for using enzyme assays or genetic testing to confirm the diagnosis prior to using Elaprase to treat MPSII can be found in a guideline published by Martin et al. Diagnosis can be confirmed by assessing the enzyme activity on cultured fibroblasts, leukocytes, plasma, or serum. Absent or low I2S activity in males is diagnostic of Hunter syndrome. Mutation analysis may be used to confirm Hunter syndrome in males. Mutations that result in complete absence of the enzyme or its activity are commonly associated with Hunter syndrome with neurologic involvement.

VII. REFERENCES

1. Elaprase [package insert]. Lexington, MA: Takeda Pharmaceuticals USA, Inc.; September 2021.
2. Muenzer J, Beck M, Eng CM, et al. Multidisciplinary management of Hunter syndrome. Pediatrics. 2009;124(6):e1228-e1239.
3. Martin R, Beck M, Eng C, et al. Recognition and diagnosis of mucopolysaccharidosis II (Hunter syndrome). Pediatrics. 2008;121(2). Available at: www.pediatrics.org/cgi/content/full/121/2/e377