Elaprase® (idursulfase)
Approved by NJ DURB, October 2019

Background:
Elaprase is a hydrolytic lysosomal glycosaminoglycan (GAG)-specific enzyme 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.

Criteria for approval:

1. Patient has documented diagnosis of Hunter syndrome (Mucopolysaccharidosis II, MPS II)
2. Diagnosis has been confirmed by one of the following:
    a. Deficient iduronate 2-sulfatase (I2S) enzyme activity present in cells (except mature red blood cells) in the presence of normal activity of at least one other sulfatase
    b. Detection of pathogenic mutations in the IDS gene by molecular genetic testing
3. Documentation of baseline values for one of the following measurements:
    a. 6-minute walk test (6-MWT)
    b. Percent predicted forced vital capacity (FVC)
    c. Spleen or liver volume for patients under 5 years of age is also acceptable
4. Prescribed by or in consultation with a physician who specializes in the treatment of inherited metabolic disorders
5. Weight must be received for drugs that have weight-based dosing. Height and weight must be received for drugs that have dosing based on body surface area.
6. 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, or national guidelines.

Initial Approval Duration: 3 months

Continuation of therapy:

1. Patient has beneficial response to therapy as compared to pretreatment baseline values in the same previously used measurement:
    a. Stabilization or improvement in 6-minute walk test (6-MWT)
    b. Stabilization or improvement in percent predicted forced vital capacity (FVC)
    c. Reduction in spleen or liver volume (for patients under 5 years of age)

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, or national guidelines.
3. For dose increases, weight must be received for drugs that have weight-based dosing. For dose increases, height and weight must be received for drugs that have dosing based on body surface area.

Renewal Approval Duration: 6 months

WARNING: RISK OF ANAPHYLAXIS

Life-threatening anaphylactic reactions have occurred in some patients during and up to 24 hours after ELAPRASE infusions. Anaphylaxis, presenting as respiratory distress, hypoxia, hypotension, urticaria and/or angioedema of throat or tongue have been reported to occur during and after ELAPRASE infusions, regardless of duration of the course of treatment. Closely observe patients during and after ELAPRASE administration and be prepared to manage anaphylaxis. Inform patients of the signs and symptoms of anaphylaxis and have them seek immediate medical care should symptoms occur. Patients with compromised respiratory function or acute respiratory disease may be at risk of serious acute exacerbation of their respiratory compromise due to hypersensitivity reactions and require additional monitoring.

References:

1. Elaprase [prescribing information]. Lexington, MA; Shire Human Genetic Therapeutics, Inc; Nov 2018
2. Giugliani R, Villareal MLS, et al. Guidelines for diagnosis and treatment of Hunter Syndrome for clinicians in Latin America. Genet Mol Biol. 2014 Jun; 37(2):315-329
3. Clinical Pharmacology® Gold Standard Series [Internet database]. Tampa FL. Elsevier 2019. Updated periodically
4. Martin R, et al. Recognition and diagnosis of Mucopolysaccharidosis II (Hunter Syndrome). Peds. 2008;121: e377-e387.
5. 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;6:72.