

PRIOR AUTHORIZATION POLICY

POLICY: Enzyme Replacement Therapy – Elaprase Prior Authorization Policy

- Elaprase® (idursulfase intravenous infusion – Shire Human Genetic Therapies)

REVIEW DATE: 04/06/2022

OVERVIEW

Elaprase, human iduronate-2-sulfatase (idursulfase), is indicated for patients with **Hunter syndrome (Mucopolysaccharidosis type II [MPS II])**.¹

Disease Overview

MPS II or Hunter syndrome, is a rare, X-linked lysosomal storage disorder characterized by a deficiency of iduronate-2-sulfatase leading to the accumulation of the glycosaminoglycans dermatan sulfate and heparin sulfate.^{2,3} Males are almost exclusively affected, although there have been a few case reports of females with Hunter syndrome.^{3,4} The onset, progression, and severity of MPS II is variable.^{2,4} Most of the patients with MPS II have a severe form with neurologic involvement leading to cognitive impairment and neurologic regression.^{3,4} Other manifestations of Hunter syndrome include coarse facial features, hepatosplenomegaly, cardiac and respiratory disease, short stature, and stiff joints and contractures.^{2,3} The definitive diagnosis of MPS II is established by demonstrating deficient iduronate-2-sulfatase activity in leukocytes, fibroblasts, serum, or plasma, or mutations in the iduronate-2-sulfatase gene.^{2,5} Definitive treatment of MPS II consists of enzyme replacement therapy with Elaprase.^{2,4} Hematopoietic stem cell transplantation has not demonstrated clear neurological benefit to date and is not recommended for MPS II due to the high rate of morbidity and mortality associated with this therapy.^{2,4}

POLICY STATEMENT

Prior Authorization is recommended for prescription benefit coverage of Elaprase. All approvals are provided for the duration noted below. Because of the specialized skills required for evaluation and diagnosis of patients treated with Elaprase as well as the monitoring required for adverse events and long-term efficacy, approval requires Elaprase to be prescribed by or in consultation with a physician who specializes in the condition being treated.

Automation: None.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Elaprase is recommended in those who meet the following criteria:

FDA-Approved Indication

- 1. Mucopolysaccharidosis Type II (Hunter Syndrome).** Approve for 1 year if the patient meets the following criteria (A and B):
 - A)** The diagnosis is established by one of the following (i or ii):
 - i.** Patient has a laboratory test demonstrating deficient iduronate-2-sulfatase activity in leukocytes, fibroblasts, serum, or plasma; OR
 - ii.** Patient has a molecular genetic test demonstrating iduronate-2-sulfatase gene mutation; AND
 - B)** Elaprase is prescribed by or in consultation with a geneticist, endocrinologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of lysosomal storage disorders.

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CONDITIONS NOT RECOMMENDED FOR APPROVAL

Coverage of Elaprase is not recommended in the following situations:

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

REFERENCES

1. Elaprase® intravenous infusion [prescribing information]. Lexington, MA: Shire Human Genetic Therapies; October 2021.
2. Scarpa M, Almassy 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.
3. Muenzer J, Beck M, Eng CM, et al. Multidisciplinary management of Hunter syndrome. *Pediatrics.* 2009;124:e1228-e1239.
4. Giugliani R, Federhen A, Munoz Rojas MV, et al. Mucopolysaccharidosis I, II, and VI: Brief review and guidelines for treatment. *Genet Mol Biol.* 2010;33:589-604.
5. D'Avanzo F, Rigon L, Zanetti A, Tomanin R. Mucopolysaccharidosis type II: One hundred years of research, diagnosis, and treatment. *Int J Mol Sci.* 2020;21:E1258.