Reference number(s)
2052-A

Specialty Guideline Management
Elaprase

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over the counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Elaprase	idursulfase

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[1]

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 are considered experimental/investigational and not medically necessary.

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Documentation

Submission of the following information is necessary to initiate the prior authorization review:

Initial Requests:

- Iduronate-2-sulfatase enzyme assay or genetic testing results supporting diagnosis.
- Chart notes or medical records documenting clinical signs and symptoms of disease at baseline (e.g., short stature, hepatosplenomegaly, joint contractures, coarse facies, frequent ear/airway infections, umbilical hernia).

Continuation Requests:

- Chart notes documenting a clinically positive response to therapy, which shall include improvement, stabilization, or slowing of disease progression (e.g., improvement, stabilization, or slowing of disease progression for predicted forced vital capacity (FVC), 6-minute walking test, global joint range of motion, combined liver and spleen volume, urinary glycosaminoglycan levels, left ventricular mass index).

Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of metabolic disease and/or lysosomal storage disorders.

Coverage Criteria

Mucopolysaccharidosis II (MPS II, Hunter syndrome)[1-3]

Authorization of 12 months may be granted for treatment of MPS II (Hunter syndrome) when both of the following criteria are met:

- The diagnosis of MPS II was confirmed by enzyme assay demonstrating a deficiency of iduronate-2-sulfatase enzyme activity or by genetic testing.
- Member exhibits clinical signs and symptoms of disease at baseline (e.g., short stature, hepatosplenomegaly, joint contractures, coarse facies, frequent ear/airway infections, umbilical hernia).

Continuation of Therapy[4]

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in the Coverage Criteria section who have a clinically positive response to therapy, which shall include improvement, stabilization, or slowing of disease progression (e.g., improvement, stabilization, or slowing of disease progression for predicted forced vital capacity (FVC), 6 minute walking test, global joint range of motion, combined liver and spleen volume, normalized urine glycosaminoglycan, left ventricular mass index).

References

1. Elaprase [package insert]. Lexington, MA: Takeda Pharmaceuticals U.S.A., Inc.; February 2025.
2. Muenzer J, Beck M, Eng CM, et al. Multidisciplinary management of Hunter syndrome. Pediatrics. 2009;124(6):e1228-e1239.
3. Scarpa M, Lampe C. Mucopolysaccharidosis Type II. 2007 Nov 6 [Updated 2025 Jan 16]. In: Adam MP, Bick S, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2026. Available from: https://www.ncbi.nlm.nih.gov/books/NBK1274/
4. Muenzer J, Wraith JE, Beck M, et al. A phase II/III clinical study of enzyme replacement therapy with idursulfase in mucopolysaccharidosis II (Hunter syndrome). *Genet Med*. 2006;8(8):465-473. doi:10.1097/01.gim.0000232477.37660.fb