CareSource Rxinnovations

PHARMACY POLICY STATEMENT
Indiana Medicaid

DRUG NAME	Elaprase (idursulfase)
BILLING CODE	J1743
BENEFIT TYPE	Medical
SITE OF SERVICE ALLOWED	Home/Office/Outpatient
STATUS	Prior Authorization Required

Elaprase is an enzyme replacement therapy that was approved by the FDA in 2006 for the treatment of Mucopolysaccharidosis type II, also known as MPS II or Hunter syndrome. MPS II is a rare, X-linked lysosomal storage disease mostly affecting males, which distinguishes it from the other MPS types which are autosomal recessive. Hunter syndrome can be classified as either severe or attenuated. Pathogenic mutations of the iduronate 2-sulfatase (IDS or I2S) gene cause the enzyme iduronate 2-sulfatase to be deficient or absent. Normally this lysosomal enzyme breaks down glycosaminoglycans (GAGs) (previously known as mucopolysaccharides) but when reduced in MPS II, the GAG substrates heparan sulfate (HS) and dermatan sulfate (DS) accumulate throughout the body causing chronic progressive damage. Elaprase has been shown to improve somatic manifestations but does not impact neurologic symptoms because it does not penetrate the blood-brain barrier. MPS I and II are the MPS types that display both somatic and neurologic symptoms. MPS I progresses faster than MPS II.

Elaprase (idursulfase) will be considered for coverage when the following criteria are met:

Hunter syndrome (Mucopolysaccharidosis II, MPS II)

For **initial** authorization:

1. Member is at least 16 months of age; AND
2. Medication must be prescribed by or in consultation with a geneticist, metabolic specialist, or pediatrician experienced with managing mucopolysaccharidoses; AND
3. Member has a diagnosis of MPS II confirmed by at least one of the following:
   a) Low iduronate 2-sulfatase enzyme activity AND normal activity of a second sulfatase (to exclude Multiple Sulfatase Deficiency), and/or
   b) Molecular genetic testing identifies pathogenic IDS gene mutation; AND
4. Documentation of baseline urinary GAG (uGAG) level; AND
5. Member does NOT have severe neurologic impairment (such as being in a vegetative state or fed by gastrostomy due to inability to swallow).
6. **Dosage allowed/Quantity limit:** 0.5 mg/kg IV infusion once weekly

If all the above requirements are met, the medication will be approved for 6 months.

For **reauthorization**:

1. Chart notes must show improvement or stabilized signs and symptoms of disease such as improved functional capacity (e.g., 6-minute walk test, forced vital capacity (FVC)) compared to baseline, reduced liver and spleen volumes, and/or reduced uGAG levels.

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Elaprase (idursulfase) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
07/22/2021	New policy for Elaprase created.

References:

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9. Nan H, Park C, Maeng S. Mucopolysaccharidoses I and II: Brief Review of Therapeutic Options and Supportive/Palliative Therapies. *Biomed Res Int*. 2020;2020:2408402. Published 2020 Dec 4. doi:10.1155/2020/2408402
10. 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;171(1):181-188. doi:10.1007/s00431-011-1606-3

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