HSE Prescribing Protocol

Idursulfase (Elaprase®)

for

Hunter Syndrome (Mucopolysaccharidosis II)

This document is intended for use by healthcare professionals only.

This guideline should be used in conjunction with the full prescribing and administration details in the Idursulfase (Elaprase®) Summary of Product Characteristics (SmPC)
https://www.ema.europa.eu/en/documents/product-information/elaprase-epar-product-information_en.pdf

INDICATION FOR USE[1]

TREATMENT	INDICATION	ICD10	PROTOCOL CODE
Idursulfase (Elaprase®)	Treatment of patients with Hunter Syndrome (Mucopolysaccharidosis II, MPS II) Heterozygous females were not studied in the clinical trials.	E76.1	ERT005

TREATMENT[1]

TREATMENT	DOSE	ROUTE	FREQUENCY	INFUSION TIME
Idursulfase (Elaprase®)	0.5 mg/kg	IV Infusion	Weekly	Run over 3 hours. This may be reduced to 1 hour if no infusion-associated reactions are observed.

This treatment should be supervised by a physician experienced in the management of patients with MPS II disease.

Infusion at home may be considered for patients who have demonstrated good tolerance to clinic-based infusions and have had no significant infusion-related reactions. Home infusions should be performed under the surveillance of a physician or other healthcare professional.

ELIGIBILITY CRITERIA

- Indication as above
- All patients must have confirmed enzymatic test, elevated urinary dermatan and heparan sulfate and/or mutation analysis confirming the diagnosis
- Documented discussion between physician and patient/caregivers regarding goals of treatment, expectation of response and criteria for discontinuing ERT
- Age-appropriate baseline assessments have been obtained
- Patients aged 0-65 years (there is no clinical experience for patients >65 years)
- Patient must attend for medical appointments and investigations as determined by the clinical team

EXCLUSION CRITERIA

- The presence of another life-threatening illness or disease where the prognosis is unlikely to be improved by enzyme replacement therapy
- The presence of severe advanced neurological disease[2,3,4] as clinically determined by physician

CONTRAINDICATIONS[1]

- Hypersensitivity to idursulfase or to any of the excipients

BASELINE TESTS AND MONITORING[5]

Once diagnosed, patients should undergo regular comprehensive assessments to evaluate the outcomes of therapy. Table 1: Recommended schedule of assessments

Assessment	Baseline	6 monthly	Annually	Periodically as indicated
Clinical history and examination	X	X		
Urine glycosaminoglycans (GAGs) dermatan and heparan sulfate	X		X	
Blood tests – FBC, LFTs, ERT antibodies if available, renal, TFTs, bone profile and vitamin D	X		X As clinically indicated	
Psychomotor assessment	X			X
Pulmonary Function Tests (where applicable)	X		X	
QoL questionnaire and pain tool	X		X	
Abdominal USS to assess liver and spleen volume	X			X
Echo and cardiac assessment	X			X As guided by cardiologist
MRI brain and spine	X			X
Ophthalmology assessment	X			X
ENT and Audiology assessment	X			X
Nerve Conduction Studies				X
6 Minute Walk Test (6MWT)	X			X

SPECIAL WARNINGS AND PRECAUTION FOR USE[1]

See SmPC for full details

STOPPING CRITERIA

- Patient is unable to tolerate infusions due to infusion related severe adverse events that cannot be resolved
- Patient is unable to comply with assessments for continued therapy
- Patient has shown no benefit to treatment after an initial 12 month period[2]
- Patients will cease to qualify for treatment if they miss more than 5 infusions in any 12 month period, excluding medical reasons for missing dosages. Missed infusions must be medically approved. No more than 2 infusions should be consecutively missed unless for a medical reason
- Coexisting illness where either long-term quality of life or expected survival is such that the patient will gain no significant benefit from idursulfase.
- Progression of neurological disease to a point where either long-term quality of life or expected survival is such that the patient will no longer gain significant benefit from idursulfase.

Patients who discontinue ERT will continue to be monitored for disease deterioration and supported with other clinical measures. These patients should continue to be assessed to allow gathering of relevant clinical information to assess a patient's on-going care needs.

ADVERSE EFFECTS[1]

See SmPC for full details

DRUG INTERACTIONS[1]

No formal medicinal product interaction studies have been conducted with idursulfase. Based on its metabolism in cellular lysosomes, idursulfase would not be a candidate for cytochrome P450 mediated interactions

ATC CODE

Idursulfase A16AB09

REIMBURSEMENT CATEGORY

ERT patients within the public health system will be funded for their treatment by the Health Service Executive (HSE). Prior funding agreement will be sought before initiation of treatment for eligible patients. Once approval for funding has been received treatment can be initiated. All new patients and dose increases for existing patients require prior approval via the HSE National Enzyme Replacement Therapy (ERT) Steering Committee. Patient applications can be made and sent to aidmp@hse.ie.

REFERENCES

1. Summary of Product Characteristics Elaprase 2mg/ml concentrate for solution for infusion. Available from: https://www.ema.europa.eu/en/documents/product-information/elaprase-epar-product-information_en.pdf
2. Muenzer J, Bodamer O, Burton B, Clarke L, Frenking GS, Giugliani R, Jones S, Rojas MV, Scarpa M, Beck M, Harmatz P. The role of enzyme replacement therapy in severe Hunter syndrome-an expert panel consensus. Eur J Pediatr. 2012 Jan;171(1):181-8.
3. Żuber Z, Kieć-Wilk B, Kałużny Ł, Wierzba J, Tylki-Szymańska A. Diagnosis and Management of Mucopolysaccharidosis Type II (Hunter Syndrome) in Poland. Biomedicines. 2023 Jun 8;11(6):1668.
4. González-Gutiérrez-Solana L, Guillén-Navarro E, Del Toro M, Dalmau J, González-Meneses A, Couce ML. Diagnosis and follow-up of patients with Hunter syndrome in Spain: A Delphi consensus. Medicine (Baltimore). 2018 Jul;97(29):e11246.
5. Expert Clinical Opinion, Consultant Paediatrician, National Centre for Inherited Metabolic Disorders.

APPENDIX

The HSE ERT Steering Committee Membership December 2025:

- Acting Chair: Carol Ivory, Assistant National Director, Specialist Acute Services, Access & Integration
- Deputy Chair: Fionnuala King, Chief Pharmacist, Access and Integration Drug Management Programme (AIDMP)
- Dr Joanne Hughes, Consultant Metabolic Paediatric Physician & Clinical Lead, National Centre for Inherited Metabolic Disorders, CHI at Temple Street
- Prof Ahmad Monavari, Consultant Metabolic Paediatrician, Clinical Director, National Centre for Inherited Metabolic Disorders, CHI at Temple Street
- Dr James O'Byrne, Consultant in Biochemical/ Clinical Genetics, National Centre for Inherited Metabolic Disorders, Mater Misericordiae University Hospital
- Dr Caoimhe Howard, Consultant Metabolic Paediatrician, National Centre for Inherited Metabolic Disorders, CHI at Temple Street
- Dr Ritma Boruah, Consultant Metabolic Paediatrician, National Centre for Inherited Metabolic Disorders, CHI at Temple Street
- Prof Ina Kerr, Consultant Metabolic Paediatrician, National Centre for Inherited Metabolic Disorders, CHI at Temple Street
- Eithne Losty, Lysosomal Storage Disorders Clinical Nurse Specialist, CHI at Temple Street
- Michael O'Keeffe, Interim ACFO, Acute Hospital Finance, HSE Finance
- Lisa Kenny, HSE Primary Care Reimbursement Service (PCRS) Representative
- Rhona O'Neill, Chief II Pharmacist, Access and Integration Drug Management Programme (AIDMP)
- Ita Quinn, Senior Pharmacist, Access and Integration Drug Management Programme (AIDMP)

HSE ERT Steering Committee Position Statement:

Patient care for ERT should be led by the centres of excellence with access to a multidisciplinary team with specialist interest in the management of patients with inherited LSDs.

Revision History

Revision Number	Revision Date	Summary of Changes