

Ontario Public Drug Programs Exceptional Access Program Elaprase (idursulfase) – Reimbursement Guidelines

Version 3 – June 2011

The Ministry will consider requests for reimbursement of Elaprase (idursulfase) for the treatment of Hunter's Syndrome/ Mucopolysaccharide (MPS) II.

Please note that patients must be eligible through the Ontario Drug Benefit (ODB) Program in order to receive coverage for these medications. Coverage is not retroactive and reimbursement may only be provided for medications dispensed after Exceptional Access Program (EAP) approval has been granted. In addition, the reimbursement criteria must always be met - even in cases where EAP approval is required to provide continued treatment that was previously supplied through a clinical trial, or paid for by other means (such as a third party payor).

Physicians may use the attached EAP request form to ensure that the necessary clinical information is provided, and to facilitate the review process. Please ensure that all relevant clinical information is provided on the request or by including copies of laboratory results. Requests should be faxed to EAP at (416) 327-7526 or toll free 1-866-811-9908.

<http://www.forms.ssb.gov.on.ca/mbs/ssb/forms/ssbforms.nsf/FormDetail?OpenForm&ACT=RDR&TAB=PROFILE&ENV=WWE&NO=014-4591-87>

Background on Ontario's Drug for Rare Diseases (DRDs) Evaluation Process for Public Drug Reimbursement

The Ontario Ministry of Health has moved forward to develop a funding framework for Drugs for Rare Diseases (DRDs). This approach recognizes that an innovative approach is required that considers the level of available clinical evidence, patient need, and the current funding gap.

In December 2007, Ontario Public Drug Programs (OPDP) established a working group comprised of clinical experts (including genetic medicine) and health economists to develop a new evaluation framework to review and evaluate DRDs for funding by the province.

The new approach is based on the "best available evidence", to assist us in predicting the potential benefit or lack of benefit of a drug treatment in specific groups of patients. This new approach will help identify groups of individuals that may potentially benefit from treatment with a particular drug, and where we may consider funding.

EAP Reimbursement Criteria:

Initial funding for Elaprase (idursulfase) under the ODB program may be considered where the patient meets the following criteria:

- Diagnosis of Hunter's Syndrome (MPS II) based on enzymology testing
- Patient is of eligible age (6 years old or older) *
- Patient has no or minimal/non-progressive neurocognitive impairment
- Patient not on chronic invasive mechanical ventilation (i.e. tracheostomy or endotracheal tube)
- Patient has no lethal IDS genetic mutation
- No other life-threatening disease where prognosis is unlikely to be influenced by Enzyme Replacement Therapy [ERT] (e.g. neuroblastoma, leukemia etc.)
- Receiving therapy under supervision of one of the provincial academic centers:
Hospital for Sick Children; Children's Hospital of Eastern Ontario; London Health Sciences Centre; McMaster Children's Hospital; or Kingston General Hospital
- If patient is to receive Elaprase treatment at home (not in a hospital) must have received Elaprase therapy for at least 1 year in an out-patient hospital setting without serious transfusion reactions prior to moving to a home setting.
- Dosage:
 - ❖ 0.5 mg/kg body weight, administered IV every week
 - ❖ Alternative dosage regimens will not be considered for reimbursement at this time

****Patients under the age of 6 years old:***

Physician should contact the manufacturer to determine whether alternative funding arrangements are available.

Extension of funding will be considered where the patient meets the following criteria:

- Patient continues to have no or minimal/non-progressive neurocognitive impairment
 - Patients 6-8 years old: Require neurocognitive testing every year
 - Patients > 8 years old: Require neurocognitive testing every 3 years

- Patient continues to not be on chronic invasive mechanical ventilation (i.e. tracheostomy or endotracheal tube)
- Patient must not be bedridden and where any physical activity brings on discomfort and symptoms which occur at rest AND not amenable to surgical/medical intervention
- No other life-threatening disease where prognosis is unlikely to be influenced by Enzyme Replacement Therapy [ERT] (e.g. neuroblastoma, leukemia etc.)
- Patient has not developed a life-threatening complication (including severe infusion-related adverse reactions) not treatable by other therapeutic measures and is unlikely to benefit from further ERT
- Patient has adhered with prescribed infusion protocol for optimal management of the disease
- Patient has adhered to all safety and effectiveness monitoring of the treatment
- Receiving therapy under supervision of one of the provincial academic centers:
Hospital for Sick Children; Children's Hospital of Eastern Ontario; London Health Sciences Centre; McMaster Children's Hospital; or Kingston General Hospital
- If patient is to receive Elaprase treatment at home (not in a hospital) must have received Elaprase therapy for at least 1 year in an out-patient hospital setting without serious transfusion reactions prior to moving to a home setting.
- Dosage:
 - ❖ 0.5 mg/kg body weight, administered IV every week
 - ❖ Alternative dosage regimens will not be considered for reimbursement at this time

If all the above renewal criteria are not met, patient may NOT be eligible for continued public funding.

Your feedback is welcomed. Please contact us at:
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