Ontario Ministry of Health and Long-Term Care

# Ontario Public Drug Programs
# Exceptional Access Program
# Myozyme (alglucosidase-alfa) – Infantile/Early Onset Pompe Disease
# Reimbursement Guidelines

Version 1 – November, 2009

The Ministry will consider requests for reimbursement of Myozyme (alglucosidase-alfa) for the treatment of **Infantile/Early Onset Pompe Disease**. Requests are assessed based on criteria established by the Ministry's expert advisory group, the Committee to Evaluate Drugs (CED), and approved by the Executive Officer for Ontario Public Drug Programs (OPDP).

The Ministry relies on a standard, rigorous review process and the CED requires data on efficacy, safety and cost-effectiveness from high quality, published randomized controlled trials. The Committee acknowledged that Pompe disease is very rare and it is therefore difficult to produce robust evidence to demonstrate clinical benefit and cost-effectiveness.

In the absence of this information, the Ministry convened a subcommittee that included clinicians with expertise in metabolic genetics, to evaluate the available evidence and develop reimbursement guidelines (with start and stop criteria) for the use of Myozyme (alglucosidase-alfa) in the treatment of Infantile/Early onset Pompe disease. The reimbursement criteria were developed in consultation with clinical experts from across the country. The subcommittee's recommendations were forwarded to the CED for consideration and endorsed.

In the absence of a pan-Canadian policy on drugs for rare diseases, the CED recommended to fund treatment based on the high fatality rate in untreated infants with early onset disease, the lack of treatment options available, and the compelling survival benefits and reduction in ventilator dependence observed in the limited clinical trial evidence.

For further information on the CED's review, please see: ***http://www.health.gov.on.ca/english/providers/program/drugs/ced_rec_table.html***

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.

**Note:** *Funding for Adult/Late onset Pompe Disease is also considered through EAP. For additional information please see:* ***http://www.health.gov.on.ca/english/providers/program/drugs/pdf/myozyme_reimbursement_adult.pdf***

**EAP Reimbursement Criteria:**

**Initial funding for Myozyme (alglucosidase-alfa) under the ODB program may be considered where the patient meets the following criteria:**

- Diagnosis of Pompe Disease based on enzymology **OR** mutational analysis; **AND** clinical features consistent with **Infantile/Early onset Pompe disease**, including:
  - Onset of generalized weakness before 12 months of age; AND
  - Feeding difficulties resulting in failure to thrive; AND
  - Presence of significant cardiomyopathy
- No other life-threatening disease where prognosis is unlikely to be influenced by Enzyme Replacement Therapy [ERT] (e.g. neuroblastoma, leukemia etc.)
- Treatment should be carried out in medical centres with expertise in the management of Infantile/Early onset Pompe disease
- Dosage:
  - 20 mg/kg body weight, administered IV every 2 weeks
  - Higher doses will not be funded
  - Requests for lower doses to be sent for clinical review by medical geneticist

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

- Patient continues to not be on chronic invasive mechanical ventilation (i.e. tracheostomy or endotracheal tube) **OR** does not deteriorate further after the initiation of ventilatory support.
- No deterioration of cardiac function (defined as left ventricular hypertrophy change of less than Z= 1 unit, OR lack of persistent clinical or echocardiographic findings of systolic or diastolic failure)
- 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 to ERT (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

- Treatment should be carried out in medical centres with expertise in the management of infantile/early onset Pompe disease

- Dosage:

    - 20 mg/kg body weight, administered IV every 2 weeks
    - Higher doses will not be funded
    - Requests for lower doses to be sent for clinical review by medical geneticist

**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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