Ontario Ministry of Health and Long-Term Care

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

Version 1 – November, 2009

The Ministry will consider requests for reimbursement of Myozyme (alglucosidase-alfa) for the treatment of Adult/Late Onset Pompe Disease.

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.

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.

Note: *Funding for Infantile/Early onset Pompe Disease has been provided since June 2007; for additional information please see:*
http://www.health.gov.on.ca/english/providers/program/drugs/pdf/myozyme_reimbursement_infantile.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 **Adult/Late onset Pompe disease**, including:
  - Significant impairment of muscle power (due to Pompe's disease) via myometric confirmation or Medical Research Council (MRC) confirmation for proximal and distal muscle; AND/OR
  - Significant impairment of respiratory function (defined as patient being on cpap/ or bipap)
- Patient not eligible under Ontario's reimbursement criteria for infantile/early onset Pompe disease
- Patient NOT on chronic invasive mechanical ventilation (i.e. tracheostomy or endotracheal tube)
- Eastern Cooperative Oncology Group (ECOG) Performance Status of grade 1-3
  http://ecog.dfci.harvard.edu/general/perf_stat.html
- 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 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 a patient falls under the following categories, the Ministry will NOT consider funding of Myozyme (alglucosidase-alfa) since it is unlikely that ERT therapy will benefit the patient in terms of disease stabilization.

- Muscle biopsy shows complete fibrosis tissue
- Patient's life expectancy less than 6 months irrespective of cause
- Patient on chronic invasive mechanical ventilation

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

- Patient's muscle power stabilizes or improves. *[if ECOG drops by 1 grade over a 6 month interval and*

is a sustained drop over 2 consecutive 6 month intervals, the Ministry should consider withdrawal of ERT therapy. Patient may not be eligible for continued reimbursement of ERT]

- Patient continues to not be on chronic invasive mechanical ventilation (i.e. tracheostomy or endotracheal tube)
- Patient must NOT be bedridden 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 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 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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