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Title **Enzyme Replacement Therapy**

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Reference Health Technology Assessment Report, MOH/P/PAK160.08 (TR), 2008.
www.moh.gov.my/MohPortal/htaDetail.jsp?action=view&id=51

Aim

To determine the effectiveness, safety, and cost implications of enzyme replacement therapy (ERT) in metabolic diseases in government hospitals.

Conclusions and results

There was sufficient evidence to indicate effectiveness and safety of enzyme replacement therapy for Gaucher type 1 (non-neuronopathic), Fabry, and Pompe (infantile) disease. ERT has not been shown to be effective for other types of Gaucher and Pompe diseases.

Recommendations

- In Malaysia, several possible options for developing treatment policies that provide explicit criteria on whether or not funding should be available for ultra-orphan drugs in the Ministry of Health by emulating the funding mechanisms of some countries in Europe, UK, and Canada.
- A National ERT Advisory Committee can be set up to evaluate each applicant for ERT. This committee should be composed of experts in metabolic disorders, consumer group representatives, and government treasury officials. Those deemed to benefit most should be prioritized for therapy. Such a prioritization process for eligibility of ERT should be in the purview of the Advisory Committee.
- Malaysia can also explore other treatment options, eg, stem cell transplantation, instead of ERT for certain rare disorders to reduce cost, although finding compatible donors is of concern.

Methods

Databases such as MEDLINE, PubMed, EBSCO, Cochrane Library, and HTA were searched from 2001 through 2007. Reference papers and cross references were accessed where applicable. Findings of papers are presented as general conclusions in some of the relevant areas involved. All relevant literature was systematically reviewed, and evidence was graded according to the modified Oxford scale.

Further research/reviews required

Further research could help clarify many uncertainties. Although doing so will be of clinical interest, it is questionable whether, within the current pricing environment, such research would have a substantive impact on policy decisions. The possible exception to this would be investigating the most efficient alternative treatment strategies for using ERT in a pediatric population.

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