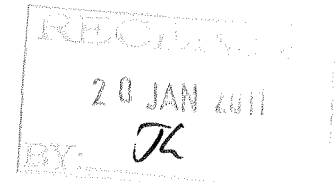


Australian Government
Department of Health and Ageing

The Hon John Murphy MP
Chair, Standing Committee on Petitions
PO Box 6012
Parliament House
CANBERRA ACT 2600



Dear Mr Murphy

Thank you for your correspondence of 25 November 2010 to the Minister for Health and Ageing, the Hon Nicola Roxon MP, regarding a petition in relation to the treatment of Adult (or late onset) Pompe disease with MYOZYME® (alglucosidase-alfa). The Minister has asked me to reply on her behalf.

The Life Saving Drugs Program provides subsidised access for eligible patients to expensive and life saving drugs for serious and rare medical conditions. There are no alternative drugs or suitable therapeutic options for the conditions treated through the LSDP.

From February 2010, MYOZYME has been subsidised through the LSDP for the treatment of infantile onset Pompe disease. MYOZYME is not currently funded through the LSDP for the treatment of late onset Pompe disease.

The Pharmaceutical Benefits Advisory Committee (PBAC) is an independent, expert advisory body comprising doctors, other health professionals and a consumer representative. In order to consider the funding of a drug through the LSDP, the drug must first be found by the PBAC to meet the LSDP funding criteria, which include that there is evidence acceptable to the PBAC to predict that a patient's lifespan will be substantially extended as a direct consequence of the use of the drug and that the drug is accepted as clinically effective, but rejected for Pharmaceutical Benefits Scheme listing because it fails to meet the required cost effectiveness criteria. The LSDP funding criteria and conditions are available at the Department's website at: www.health.gov.au/lstdp.

The PBAC has not recommended the funding of MYOZYME through the LSDP for the treatment of late onset Pompe disease. The PBAC has considered submissions on four occasions (July 2008, March, November 2009 and November 2010) and on each occasion found that there was insufficient evidence to demonstrate that a patient's lifespan will be extended as a direct consequence of treatment with MYOZYME. The PBAC found that there is still considerable uncertainty remaining around the magnitude of benefit gained through treatment with this product, and the extent to which MYOZYME can be reasonably expected to prolong life for late onset Pompe patients.

The outcomes of the November 2010 PBAC meeting were released on 17 December 2010 and are enclosed for your information.

In addition, a Public Summary Document containing full details of the PBAC decision will be available from the Department of Health and Ageing web site 16-18 weeks after the PBAC meeting at: <http://www.health.gov.au/internet/main/publishing.nsf/Content/public-summary-documents>.

I trust that this information is of assistance.

Yours sincerely

David Reddy
A/g Assistant Secretary
Access and Systems Branch
Pharmaceutical Benefits Division

20 December 2010

Encl.

(1) PBAC meeting outcomes, MYOZYME for late onset Pompe disease, November 2010