Senate Community Affairs Committee

ANSWERS TO ESTIMATES QUESTIONS ON NOTICE

HEALTH AND AGEING PORTFOLIO

Budget Estimates 2013-14, 5/6 & 7 June 2013

Question: E13-131

OUTCOME: 2 – Access to Pharmaceutical Services

Topic: Use of Evidence

Type of Question: Written Question on Notice

Senator: Boyce

Question:

The Erasmus University in the Netherlands has recently published an international study demonstrating the benefits of Myozyme.

a) Given the small population group of Pompe disease sufferers in Australia, does the PBAC take into consideration overseas research when it comes to rare diseases? If not why not?

b) Have companies who have had made application under the LSDP, been told by the Chair of the PBAC to apply to the PBS at a much lower price?

c) Does that not suggest that the PBAC recognises that Myozyme benefits Pompe disease sufferers?

d) Has the Department had discussions with the PBAC about the fact that "late-onset" Pompe disease includes children between the ages of 2 and 18?

e) Has any consideration been given to making an exception for these children?

f) Are you aware that those with no access to treatment include an 11 year old boy whose condition is worsening due to the fact that the application for treatment has been rejected by the PBAC?

g) Have discussions been held between the Department and the PBAC in respect of the exclusion of late onset patients on the grounds of cost?

h) If so, when?

i) Are you aware that the price for this medication is based on the cost of a standard vial price and that dosage for patients and therefore cost is weight related?

Answer:

a) Yes.

b) and c)
The Pharmaceutical Benefits Advisory Committee (PBAC) has provided the sponsor of Myozyme with a possible way forward if it wishes to further apply for Government subsidy. This involves a possible application for listing of Myozyme on the Pharmaceutical Benefits Scheme (PBS) under rule of rescue criteria, but at a significantly lower drug cost per patient per year for treating late-onset Pompe disease than proposed in the March 2013 submission. The PBAC rejected Myozyme for late-onset Pompe disease through the LSDP in March 2013 because although the medicine may have some effect on mortality and quality of life, the available data do not provide certainty to the PBAC that the drug substantially extends the lifespan of patients with late-onset Pompe disease. Further information on the PBAC's decision can be found through the Public Summary Document on the PBS website at www.pbs.gov.au/info/industry/listing/elements/pbac-meetings/psd/2013-03/alglucosidase

d) and e)
The data provided in submissions by the sponsor for PBAC consideration to support the treatment of late-onset Pompe disease included children between the ages of two and 18 years of age. The data provided by the sponsor, however, was limited and insufficient for the PBAC to make a positive recommendation for the treatment of this patient cohort through the Life Saving Drugs Program (LSDP). The data may, however, support the listing of Myozyme on the PBS under rule of rescue criteria, but at a significantly lower drug cost per patient per year for treating late-onset Pompe disease than proposed in the most recent submission.

f) Yes.

g) and h)
The PBAC is an independent, expert advisory body comprising doctors, other health professionals and a consumer representative. The PBAC considers submissions having regard to the safety, clinical effectiveness and cost-effectiveness (value-for-money) of the medicine for the intended use, in comparison with other available treatments. In line with these considerations, the PBAC makes recommendations to the Minister for Health and Ageing about funding of medicines.

i) Yes, that is the pricing approach proposed by the sponsor. However, in March 2013, the PBAC considered that at the price requested, Myozyme for late-onset Pompe disease appears to be less effective and more expensive (per patient per year) than Myozyme for infantile-onset Pompe disease patients. The PBAC advised that, in this specific circumstance, the cost per treatment should not be based on individual patient's weight, but on a determined maximum cost per patient per year.

   Refer also to the answer provided to part b).