Senate Community Affairs Committee

ANSWERS TO ESTIMATES QUESTIONS ON NOTICE

HEALTH AND AGEING PORTFOLIO

Additional Estimates 13 & 15 February 2013

Question: E13-050

OUTCOME 2: Access to Pharmaceutical Services

Topic: Myozyme, a life saving drug used to treat Pompe Disease

Type of Question: Written Question on Notice

Senator: Senator Boyce

Question:

Is the level of evidence being required for the treatment of Pompe's disease different from that which was required for other treatments on the Life Saving Drugs program? If so, what has changed and why?

Answer:

The Life Saving Drugs Program (LSDP) currently funds ten medicines for seven rare conditions. In 2011-12, 215 patients were treated through the LSDP at a cost of $77.8 million.

Unlike all drugs supplied through the Pharmaceutical Benefits Scheme, drugs supplied through the LSDP are not cost-effective, therefore other evidence of benefit must be provided to justify funding under a life saving program.

The most recent review of the program was in 2009. Revised LSDP Criteria and Conditions for Funding became effective on 10 May 2010.

A key component of the amendments to the LSDP Criteria and Conditions for Funding was that the submission of evidence to predict that a patient's lifespan will be substantially extended as a direct consequence of the use of the drug is now required.

Myozyme® (alglucosidase alfa) was recommended for the treatment of patients with infantile-onset Pompe disease in July 2008 and since February 2010, the Government has funded Myozyme treatment for these patients.

The PBAC considered submissions to fund Myozyme through the LSDP for the treatment of late-onset Pompe disease against the former LSDP Criteria and Conditions for Funding in July 2008, March 2009, November 2009 and against the amended Criteria and Conditions for Funding in November 2010, July 2011 and November 2012 without recommending funding through the LSDP.

The Pharmaceutical Benefits Advisory Committee again considered and rejected the funding of Myozyme for late-onset Pompe disease at its March 2013 meeting. Outcomes from the meeting were available from 26 April 2013 on the PBS website at www.pbs.gov.au/info/industry/listing/elements/pbac-meetings/pbac-outcomes

Since the LSDP Criteria and Conditions for Funding were amended in 2010, the following drugs were recommended for funding through the LSDP and received Government approval: Soliris® (eculizumab) for the treatment of Paroxysmal Nocturnal Haemoglobinuria and VPRIV® (velaglucerase) for the treatment of Gaucher disease.

Further information about the review of the LSDP in 2009 is available on the Department of Health and Ageing website at www.health.gov.au/internet/main/publishing.nsf/Content/lsdp-preview

Further information on the revised criteria for the funding of a medicine through the LSDP is available on the Department of Health and Ageing's website at www.health.gov.au/internet/main/publishing.nsf/Content/lsdp-criteria