Senate Community Affairs Committee

ANSWERS TO ESTIMATES QUESTIONS ON NOTICE

HEALTH AND AGEING PORTFOLIO

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

Question: E13-129

OUTCOME: 2 – Access to Pharmaceutical Services

Topic: Access to Myozyme

Type of Question: Written Question on Notice

Senator: Boyce

Question:

a) Is it true that the Pharmaceutical Benefits Advisory Committee (PBAC) has rejected Myozyme® a total of seven times for Late Onset Pompe Disease in adults?

b) Is it true that the Life Saving Drug Program (LSDP) criteria were altered so that Criterion Four (4) was worded to include the word substantial?

c) When was the criteria changed?

d) How many drugs have been approved since the change in the criteria?

e) What is the definition of "substantial"?

f) How can "substantial" be measured when it can mean different things for different diseases?

g) Is it not a nebulous term which introduces an unacceptable amount of uncertainty into the guidelines and undermines the whole program?

h) Is criterion 4 in its changed form an unrealistic, impossible and uncompassionate provision given that in these ultra-rare diseases, clinical data simply cannot be provided at the level available for common diseases and no-one seems to be clear on what a "substantial" life extension actually means?

i) If the manufacturer of the drug - Genzyme - had made application under the old LSDP guidelines, would the drug have been approved?

Answer:

a) The Pharmaceutical Benefits Advisory Committee (PBAC) considered submissions to fund Myozyme® through the Life Saving Drugs Program (LSDP) for the treatment of late-onset Pompe disease on seven occasions, without recommending funding through the LSDP. The submissions were rejected in July 2008, March 2009, November 2009, November 2010 and November 2012, while the July 2011 submission was deferred. In March 2013, the PBAC considered that the sponsor may wish to consider a possible

application for listing of Myozyme on the Pharmaceutical Benefits Scheme 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.

b) Yes.

c) Following a public consultation process, revised LSDP Criteria and Conditions for Funding became effective on 10 May 2010.

d) Two medicines have been listed through the LSDP since the criteria were revised. Soliris® (eculizumab) for the treatment of Paroxysmal Nocturnal Haemoglobinuria became available through the LSDP from 1 January 2011 at a cost of approximately $135 million over five years and VPRIV® (velaglucerase) for the treatment of Gaucher disease became available through the LSDP from 1 August 2012.

e), f) and g)
Each individual application is assessed on its merit based on the strength of the clinical evidence which is submitted by the pharmaceutical company, tailored to the specific disease.

h) No. Refer to answer provided to part d).

i) The sponsor of Myozyme, Genzyme, made three submissions for the funding of late onset Pompe disease under the previous LSDP Criteria and Conditions for Funding, none of which were successful.