

Senate Community Affairs Committee

ANSWERS TO ESTIMATES QUESTIONS ON NOTICE

HEALTH AND AGEING PORTFOLIO

Additional Estimates 13 & 15 February 2013

Question: E13-044

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:

In estimates last week, the department said that the Pharmaceutical benefits advisory Committee last year deferred their decision on listing Myozyme on the Life Saving Drugs program, when in fact it was rejected again at the November PBAC meeting. You also stated that consideration would be given to observational data from a small clinical group of patients to prove the effectiveness or otherwise of Myozyme. It is my understanding that the company has already provided you with data from a global observational study, which includes Australian data, is this correct? What else do you require from the company?

Answer:

At its July 2011 meeting, the Pharmaceutical Benefits Advisory Committee (PBAC) deferred its decision seeking further data from the sponsor to allow the PBAC to have confidence that a patient's life would be substantially extended as a direct consequence of the use of Myozyme®.

At its November 2012 meeting, the PBAC rejected the application "on the basis of uncertainty about whether the lifespan of a patient with late-onset Pompe disease would be substantially extended as a direct consequence of using Myozyme".

The Public Summary Document for these decisions are available on the Department of Health and Ageing's Pharmaceutical Benefits Scheme website at:

- <http://www.health.gov.au/internet/main/publishing.nsf/Content/pbac-psd-mtg-july-2011>
- <http://pbs.gov.au/info/industry/listing/elements/pbac-meetings/psd/2012-11>

The sponsor resubmitted a further application for the PBAC to consider at its March 2013 meeting.

The outcomes of the March 2013 meeting of the PBAC will become public on 26 April 2013.

Submissions provided to the PBAC are commercially sensitive.