Senate Community Affairs Committee

ANSWERS TO ESTIMATES QUESTIONS ON NOTICE

HEALTH AND AGEING PORTFOLIO

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

Question: E13-130

OUTCOME: 2 – Access to Pharmaceutical Services

Topic: Pharmaceutical Benefits Advisory Committee Decision Making

Type of Question: Written Question on Notice

Senator: Boyce

Question:

The grounds the PBAC gave for declining Myozyme in March 2013 was that it did not meet criterion 4 of the LSDP, in that although Myozyme may have some effect on mortality and quality of life, the available data do not provide certainty to the PBAC that the drug substantially and directly extends the lifespan of patients with late-onset Pompe disease.

a) In Australia, there are 29 patients diagnosed with late onset Pompe disease. Will a population group this size ever be large enough to provide that certainty?

b) Is the PBAC requiring evidence that simply cannot be provided in Australia?

c) If it requires evidence that cannot be produced, is that not defeating the whole purpose of the LSDP whose ambit is to assist small populations of people with rare diseases?

Prof Jack Goldblatt, a leading Australian geneticist and metabolic physician, recently concluded that the interpretation of the LSDP criterion in the PBAC's decision on treatment for Pompe disease suggests this criterion will likely mitigate against new drugs for these rare disorders being funded through the LSDP in the future. He went on to say that this vulnerable group of individuals will thus potentially be denied therapy for conditions which will progress to premature death if untreated.

d) That is the view of an expert. Is the PBAC not breaching its own guidelines by denying Pompe disease sufferers' treatment which, according to research, improves and extends their lives?

Answer:

a), b) and c)
The listing of all medicines on the Pharmaceutical Benefits Scheme (PBS) and through the Life Saving Drugs Program (LSDP) is underpinned by evidence based decision making. When evaluating submissions, evidence provided by the Sponsor Company in the form of published and unpublished clinical studies as well as evidence from observational studies conducted in Australia and internationally and literature is considered by the Pharmaceutical Benefits Advisory Committee (PBAC) to inform decisions. For example, data from international studies was used to support the 2010 submission for the listing of eculizumab on the LSDP for Paroxysmal Nocturnal Haemoglobinuria, which received a positive recommendation.

The size of the patient population in Australia is usually included in the submission to provide information about the usage of the drug in Australia and to calculate budget impacts but does not directly affect the quality of the clinical evidence required, because the studies presented by the Sponsor comprise all the data available to date whether conducted here or overseas.

d) Unlike all drugs supplied through the PBS, 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. Therefore, it is up to the Sponsor Company to ensure that sufficient evidence is made available in the submission to convince the PBAC that for a drug that is very expensive and cannot demonstrate cost effectiveness, a patient's lifespan will be substantially extended as a direct consequence of the use of that drug.