Senate Community Affairs Committee

ANSWERS TO ESTIMATES QUESTIONS ON NOTICE

HEALTH PORTFOLIO

Budget Estimates 2014 - 2015, 2/3 June 2014

Ref No: SQ14-000402

OUTCOME: 2 - Access to Pharmaceutical Services

Topic: Soliris

Type of Question: Written Question on Notice

Senator: Madigan, John

Question:

a) What is the status of Government-funded access to Soliris for those patients who most urgently need it?
b) Why was Soliris recommended for listing under the Section 100 Highly Specialised Drugs Program - with difficult conditions attached instead of to the Life Saving Drugs Program (LSDP) where it met the criteria?
c) Why wasn't it recommended to the Life Savings Drug Program? If it did not meet the LSDP criteria, which aspects of the criteria specifically did it not meet?
d) Have you closed the LSDP to new listings while the program is being reviewed? Is this why Soliris wasn't recommended for listing under the Program?

Answer:

a) The Australian Government has subsidised Soliris® (eculizumab) through the Life Saving Drugs Programme (LSDP) for the treatment of paroxysmal nocturnal haemoglobinuria since 1 January 2011.

In March 2014, in response to an application by the sponsor, Alexion Pharmaceuticals Australasia Pty Ltd, the Pharmaceutical Benefits Advisory Committee (PBAC) recommended the Pharmaceutical Benefits Scheme (PBS) listing of Soliris for the treatment of Atypical Hemolytic Uremic Syndrome (aHUS) under a Managed Entry Scheme arrangement.

PBAC considered, that despite the extremely high price requested, the medicine could be cost-effective under the PBS if the sponsor rebated part or all of the price of the drug when used in patients where it is shown to be less effective or ineffective. This means that all eligible patients could be treated with PBS-subsidised Soliris but the price paid by Government should vary depending on the magnitude of the benefit gained by patients.

Negotiations between the Department of Health and Alexion Pharmaceuticals Australasia Pty Ltd commenced soon after the PBAC meeting to progress the PBAC's recommendation.

A stakeholder meeting was held on 24 June 2014. All invited stakeholders participated, including members of PBAC, nephrologists, haematologists, patients and the pharmaceutical company. The organisations that were represented included the Australian and New Zealand Paediatric Nephrology Association, the Haematology Society of Australia and New Zealand, Kidney Health Australia, the aHUS Patient Support Group Australia, Rare Voices Australia, Alexion, the Department of Health and the Department of Human Services.

There was constructive discussion that worked towards finalising the prescribing criteria for listing the product on the PBS.

The Department of Health will continue to work with the sponsor to finalise risk sharing arrangements consistent with the PBAC recommendation from March 2014 and the clinical criteria worked through at the stakeholder meeting, recognising the high and unmet clinical need for an effective treatment for patients with aHUS.

A summary of the meeting will be published on the PBS website in the coming weeks.

b) and c)
A medicine can only be considered for inclusion through the LSDP if it does not meet the cost-effectiveness criteria for PBS listing. Only if a medicine has been rejected for PBS subsidy upon that basis, the PBAC may then assess it for inclusion through the LSDP against the LSDP criteria.

The sponsor of Soliris, Alexion Pharmaceuticals Australasia Pty Ltd, made a re-submission to request Section 100 (Highly Specialised Drugs Program) or LSDP listing for aHUS for PBAC's consideration at its March 2014 meeting. The PBAC recommended the PBS listing of Soliris for aHUS through special arrangements under Section 100 of the *National Health Act 1953*.

d) No. The post-market review of the LSDP does not affect patients who are currently treated through the LSDP - they will continue to receive access to treatment. New patients may continue to apply for access to treatment and applications for new medicines seeking listing will continue to be considered throughout the review period.