Recommendations made by the PBAC – August out-of-session meeting

DRUG, SPONSOR, TYPE OF SUBMISSION	DRUG TYPE OR USE	LISTING REQUESTED BY SPONSOR	PBAC RECOMMENDATION
ECULIZUMAB 300 mg/30 mL injection, 1 x 30mL vial Soliris® Alexion Pharmaceuticals Australasia Pty Ltd New listing	Atypical Haemolytic Uraemic Syndrome (aHUS)	Re-submission following the PBAC's March 2014 recommendation to list eculizumab on the PBS for the treatment of aHUS.	The PBAC noted that following its March 2014 recommendation to list eculizumab, the Department of Health had developed PBS restrictions taking account of the "managed entry scheme" and the initiation and continuation criteria that the Committee had recommended. The PBAC further noted that the Department had developed these restrictions in an iterative fashion following consultation with clinicians, patients, patient advocates and the sponsor. The sponsor had proposed alternative continuation criteria. The purpose of the out-of–session PBAC meeting was to provide advice regarding the proposed initiation and continuation criteria, particularly regarding the duration of the initial period of PBS subsidised treatment, and the criteria for restarting PBS-subsidised therapy. The PBAC considered that no convincing evidence was presented that demonstrated that all patients should routinely have uninterrupted, life-long eculizumab therapy. The PBAC re-iterated its advice from March 2014 that for patients who are able to demonstrate a response to the point that they achieve remission, it would be reasonable for PBS-subsidised treatment to discontinue after a defined period given that eculizumab is not without side effects. Clinical progress would be monitored and the need for further treatment assessed, with reintroduction of eculizumab enabled without prejudice if the disease relapses. Further, the PBAC concluded that, in light of the evidence currently available, the most appropriate duration for initial, continuous administration of eculizumab is 12 months. In reaching this conclusion the PBAC noted, among other matters, that the vast majority of the benefit observed in patients receiving eculizumab occurs in the first 6 months of treatment. The PBAC concluded that patients with limited organ reserve should be eligible to receive continuing eculizumab treatment, because these patients would have the least capacity to recover from a subsequent aHUS attack. In making this recommendation, the PBAC aimed to strike an appropriate balance between clinical need, patient safety and cost-effectiveness in the context of the current evidence base. The PBAC considered that there were many areas in which evidence is likely to emerge rapidly, and it would welcome the provision of such data for the Committee's consideration as it becomes available.