November 2007 PBAC Outcomes – Subsequent decisions not to recommend

Drug name and Sponsor	TGA Indication	Current PBS Listing	Listing Requested by Sponsor	PBAC Outcome and Comments
Darbepoetin alfa, pre-filled syringe, 200 mcg in 0.4 mL, 300 mcg in 0.6 mL and 500 mcg in 1 mL, Aranesp®, Aranesp SureClick®, Amgen Australia Pty Ltd Major submission	Darbepoetin alfa is indicated for the treatment of anaemia associated with chronic renal failure (CRF). Darbepoetin alfa is also indicated for the treatment of anaemia and reduction of transfusion requirements in patients with non-myeloid malignancies where anaemia develops as a result of concomitantly administered chemotherapy.	<u>Section 100 Highly Specialised Drug</u> <u>Private hospital authority required</u> Treatment of anaemia requiring transfusion, defined as a haemoglobin level of less than 100g per L, where intrinsic renal disease, as assessed by a nephrologist, is the primary cause of the anaemia.		PBAC rejected the submission because of uncertain cost-effectiveness.
			<u>Section 100 Highly Specialised Drug</u> <u>Private hospital authority required</u> Treatment of chemotherapy induced anaemia in patients with non-myeloid malignancies who satisfy all of the following criteria: (1) Haemoglobin level of less than 110 g/L OR 100 g per L (2) At risk of requiring transfusion; (3) Scheduled to receive at least a further 12 weeks of chemotherapy; (4) Adequate iron stores as defined by iron studies.	PBAC considered the haemoglobin level at which therapy should commence should be set at 100 g/L, consistent with the NHMRC guidelines for transfusion. Also 'at risk of requiring transfusion' and 'adequate iron stores' should be more clearly defined.
			Comparator: Placebo or no pharmacological treatment.	Accepted

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			Clinical claim: Darbepoetin alfa is significantly more effective than the main comparator (placebo/no pharmacological treatment) and has similar or less toxicity.	The trial data showed no improvement in quality of life (QoL) or survival compared with blood transfusion, but showed a statistically significant reduction in the proportion of patients requiring transfusion, and the number of units transfused per patient. Although there may be long term risks associated with blood transfusion, these may be of a lesser concern to patients whose life expectancy is shortened due to their cancer than improvement in QoL. Studies of patient populations outside the registered chemotherapy-induced anaemia population, and also outside the requested PBS population, have cause concerns regarding tumour progression, survival and cadiovascular/thrombotic events.
			Economic claim: Cost-effectiveness	Not accepted. In the absence of a survival benefit or significant improvement in QoL for patients, the incremental cost-effectiveness ratios per patient avoiding transfusion and per transfusion avoided were considered unacceptably high.

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			Sponsor's comments	Although disagreeing with the recent recommendation, the Sponsor intends to work collaboratively with the PBAC to find a way to move forward with reimbursement of darbepoetin alfa for the treatment of chemotherapy-induced anaemia.