JULY 2004 PBAC OUTCOMES - "Subsequent" Decisions not to Recommend

DRUG AND FORM	TGA INDICATION	CURRENT PBS LISTING	LISTING REQUESTED BY SPONSOR	PBAC OUTCOME AND COMMENTS
FENTANYL citrate lozenges with integral applicators, 200 µg, 400 µg, 600 µg, 800 µg, Actiq® Orphan Australia Pty Ltd	For management of breakthrough cancer pain in patients with malignancies who are already receiving and are tolerant to opioid therapy for their underlying persistent cancer pain.	Not PBS listed		PBAC rejected the application because of uncertain extent of clinical benefit and the resulting uncertain and unfavourable cost effectiveness.
			Preparations which may be prescribed for patients receiving palliative care <u>Authority required</u> **Initiation** by a specialist in cancer pain management and ongoing therapy by a specialist in cancer pain management, or where consultation with a specialist in cancer pain management or palliative care service for management of breakthrough cancer pain in palliative care patients who are receiving opioids for their underlying persistent cancer pain and where morphine and one other opioid are each precluded from use.	Accepted, subject to minor changes for clarification.
			Comparator: Placebo	Accepted

			Clinical claim: Fentanyl lozenges produce greater pain relief compared to placebo, but have more toxicity.	Partially accepted. PBAC considered that the evidence base available is limited and the extent of clinical benefit remains unclear from the data provided. The key trial had a cross-over design and included patients who were not directly representative of those for whom PBS listing is sought. Further, the observed placebo effect in the key trial was substantial.
			Economic claim: A cost-effectiveness analysis was presented.	Rejected. Based on the data provided and the relatively short-term nature of breakthrough pain, the interpretation of the incremental cost-effectiveness ratio is difficult and associated with uncertainty.
			Sponsor's comments:	The sponsor will be considering its position regarding any future course of action.

INTERFERON BETA-1A 30 µg vials lyophilised powder, plus pre-filled syringe of diluent 2 mL water for injection, Avonex® Biogen Idec Australia Pty Ltd	Relapsing forms of multiple sclerosis. In patients who have experienced a single demyelinating event and are at risk of developing clinically definite multiple sclerosis based on the presence of brain MRI abnormalities characteristic of MS.	PBS listed for certain patients with the relapsing-remitting form of multiple sclerosis.		PBAC rejected the submission because of uncertain clinical benefit and the resulting uncertain and unfavourable cost-effectiveness.
			Extend Authority required listing to include treatment of patients who experience a demyelinating clinical event with an active inflammatory process if the following conditions are satisfied: (i) the event is severe enough to warrant treatment with intravenous corticosteroids; (ii) alternative diagnoses have been excluded; and (iii) the patient is determined to be at high risk of developing clinically definite multiple sclerosis within 2 years based on the presence of ≥ 1 Gd-enhanced lesion and ≥ 9 T2 lesions on magnetic resonance imaging (MRI) taken no more than 3 months after the clinical event.	Rejected. PBAC expressed doubt over whether the evidence provides an adequate basis to define a high-risk patient group.
			Comparator: Placebo	Accepted

			Clinical claim: Treatment benefits of Avonex in high risk clinically isolated syndrome patients are superior to those of placebo.	Partially accepted. PBAC noted that the relationship between clinically definite multiple sclerosis and more patient-relevant outcomes remains unclear. PBAC considered there is not any clear evidence to support its argument that early intervention can alter long-term outcomes of the disease.
			Economic claim: No economic claim was made	It is uncertain whether targeting Avonex to the high-risk group proposed in the requested restriction is likely to improve the extent of benefit of Avonex, sufficiently to reduce the previously unacceptable incremental cost-effectiveness ratios to acceptable levels.
			Sponsor's comments:	The sponsor disagrees with the decision.