NOVEMBER 2009 PBAC MEETING OUTCOMES – "Subsequent" decisions not to recommend

DRUG AND FORM	TGA INDICATION	CURRENT PBS LISTING	LISTING REQUESTED BY SPONSOR	PBAC OUTCOME AND COMMENTS
Alglucosidase alfa, powder for I.V. infusion, 50 mg, (Myozyme® Genzyme Australasia Pty Ltd Major submission	Long-term treatment of patients with a confirmed diagnosis of Pompe disease (acid alfa-glucosidase deficiency).	Not currently listed.		The PBAC rejected the re-submission to list alglucosidase on the PBS as a Section 100 Highly Specialised Drug for the treatment of patients with late onset Pompe disease on the basis of an unacceptably high cost effectiveness ratio compared to standard (palliative) therapy. The PBAC did not recommend inclusion in the LSDP of alglucosidase for the treatment late onset Pompe disease considering that insufficient evidence was provided to demonstrate that a patient's lifespan will be extended as a direct consequence of treatment with alglucosidase, and hence that criterion two of the LSDP had not been met.
			Section 100 (Highly Specialised Drug) <u>Private hospital authority required</u> Patients with a confirmed clinical diagnosis of Pompe disease who have had their diagnosis confirmed by a documented deficiency of alfa-glucosidase enzyme activity in either skin fibroblasts, muscle tissue, lymphocytes, mixed leukocytes or dried blood spots (< 40% of normal levels) or through identification of a mutation in the GAA gene and who meet the criteria below: <u>Inclusion criteria:</u> Patients with significant deterioration in either: Lung function: FVC in upright position of < 80% predicted; or Muscle function: clinically significant muscle weakness. <u>Exclusion criteria:</u> Patients with chronic invasive ventilation of >12 months duration. OR The treatment of a patient with late-onset Pompe disease who meets certain criteria to be considered for inclusion in the Life Savings Drugs Program (LSDP).	

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			Comparator: Standard (palliative) therapy including intensive respiratory support, cardiac care, dietary therapy and rehabilitative services.	Accepted (as previously)
			Clinical Claim: Alglucosidase is superior in terms of comparative effectiveness and inferior in terms of comparative safety over placebo (standard management).	Partially accepted. The PBAC accepted that alglucosidase therapy is associated with an improvement in the 6-minute walk test (6MWT) and lung function compared with placebo. However, the PBAC considered that there was uncertainty in the extrapolation to survival gain from the evidence presented based on surrogate outcomes.
			Economic Claim: Cost-effectiveness	Not accepted. The incremental cost-effectiveness ratio remained unacceptably high.
			Sponsor Comments:	Genzyme Australasia will work with the PBAC and the LSDP with a continued sense of urgency to ensure that people with late-onset Pompe disease have funded access to Myozyme.
Diclofenac sodium with misoprostol, tablet, 50 mg – 200 micrograms, Arthrotec®	The treatment of patients who require a non-steroidal anti-inflammatory drug (NSAID) together with misoprostol. The diclofenac component of Arthrotec 50 is indicated for the treatment of osteoarthritis and rheumatoid arthritis. The misoprostol component of Arthrotec 50 is indicated for the prophylaxis of NSAID induced gastric and duodenal ulceration. Known risk factors for NSAID induced gastropathy include	Not currently listed.		The PBAC rejected the submission on the basis of lack of clinical need and the potential for unwanted clinical outcomes.
Pfizer Australia Pty LTd Major submission			<u>Authority Required (STREAMLINED)</u> Osteoarthritis or rheumatoid arthritis in patients who require prophylaxis against NSAID-induced peptic ulcers. <u>NOTE:</u> Known risk factors include age in excess of 60 years, a history of peptic ulcer disease, smoking, previous NSAID gastrointestinal intolerance, and serious co-morbid disease.	The PBAC was concerned the patient group targeted was inappropriate given the cardiovascular toxicity associated with diclofenac.

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			Comparator: Diclofenac and misoprostol, the individual components of the fixed combination (major). NSAID in combination with a Proton Pump Inhibitor (PPI) (secondary)	Accepted.
			Clinical Claim: Diclofenac with misoprostol is non-inferior to diclofenac monotherapy for arthritis outcomes; and is non-inferior to both concomitant diclofenac and misoprostol and combination NSAID and PPI with respect to the reduction in NSAID-induced gastrointestinal complications endpoints.	Not accepted. The PBAC considered the trial evidence does not conclusively prove the combination product is non-inferior to concomitant diclofenac and misoprostol, and combination NSAID and PPI for the reduction of NSAID-induced ulcers.
			Economic Claim: Cost minimisation based on cost per mg of the individual components.	Not accepted, because the clinical claim was not accepted.
			Sponsor Comments:	The Sponsor has no comment.