

MARCH 2018 PBAC OUTCOMES – DEFERRALS

DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION	DRUG TYPE AND USE	LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION	PBAC OUTCOME
<p>ADRENALINE</p> <p>I.M. injection 150 micrograms in 0.3 mL single dose syringe auto-injector</p> <p>I.M. injection 300 micrograms in 0.3 mL single dose syringe auto-injector</p> <p>AdrenaJect®</p> <p>Sun Pharma ANZ Pty Ltd</p> <p>Change to listing (Minor Submission)</p>	<p>Anaphylaxis</p>	<p>To request that the PBAC reconsider its previous recommendation that AdrenaJect not be considered equivalent for the purposes of substitution with the reference originator.</p>	<p>The PBAC deferred making a recommendation on the brand equivalence of AdrenaJect® with the originator brand, EpiPen®, for the purposes of brand substitution ('a' flagging) at the pharmacy level. The PBAC requested further information from the Sponsor regarding their education plan to support the PBS listing of AdrenaJect®. The PBAC also requested further information from the Department regarding possible alternative measures that could support safe uptake.</p>
		<p>Sponsor Comment:</p>	<p>The sponsor had no comment.</p>
<p>AMINO ACID FORMULA WITH VITAMINS, MINERALS AND LONG CHAIN POLYUNSATURATED FATTY ACIDS, WITHOUT PHENYLALANINE</p> <p>Oral powder 400 g (PKU Start)</p> <p>PKU Start®</p> <p>Vitaflo Australia Pty Ltd</p> <p>New listing (Minor Submission)</p>	<p>Phenylketonuria</p>	<p>To request a Restricted Benefit listing for the dietary management of patients with phenylketonuria.</p>	<p>The PBAC decided to defer its decision on whether to recommend the listing of amino acid formula with vitamins, minerals and long chain polysaturated fatty acids, without phenylalanine for the dietary management of Phenylketonuria (PKU) due to the concerns about the sufficiency of the iron level. The PBAC requested advice from the Australasian Society for Inborn Errors of Metabolism regarding the clinical need for the requested listing. The PBAC considered that this advice should be considered by the Nutritional Products Working Party (NPWP) prior to the PBAC finalising its decision.</p>
		<p>Sponsor Comment:</p>	<p>The sponsor appreciates the opportunity to provide input into this matter.</p>

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<p>FILGRASTIM</p> <p>Injection 120 micrograms in 0.2 mL single use pre-filled syringe Injection 300 micrograms in 0.5 mL single use pre-filled syringe Injection 480 micrograms in 0.5 mL single use pre-filled syringe</p> <p>Nivestim®</p> <p>Pfizer Australia Pty Ltd</p> <p>Change to listing (Minor Submission)</p>	<p>Chemotherapy-induced neutropenia; Mobilisation of peripheral blood progenitor cells; Assisting bone marrow transplantation; Assisting autologous peripheral blood progenitor cell transplantation; Severe congenital neutropenia; Severe chronic neutropenia; Chronic cyclical neutropenia</p>	<p>To request that the current listings of Nivestim be changed to Authority Required (STREAMLINED) for Section 100 Highly Specialised Drugs (Private Hospital).</p> <p>Sponsor Comment:</p>	<p>The PBAC deferred making a recommendation on this submission to seeking further information regarding whether the four filgrastim brands currently PBS listed could be marked as equivalent in the Schedule of Pharmaceutical Benefits ('a' flagged), allowing substitution by the pharmacist at the point of dispensing for all circumstances for which filgrastim is listed. The PBAC, in principle, considered that filgrastim is suitable for the application of biosimilar uptake drivers for all biosimilar brands. However, the PBAC decided to defer making a recommendation regarding application of the biosimilar uptake drivers at this time.</p> <p>The sponsor had no comment.</p>
<p>GRAZOPREVIR + ELBASVIR</p> <p>Tablet containing grazoprevir 100 mg with elbasvir 50 mg</p> <p>Zepatier™</p> <p>Merck, Sharp and Dohme Australia Pty Limited</p> <p>Change to listing (Minor Submission)</p>	<p>Chronic Hepatitis C virus (HCV) infection</p>	<p>To request that the current General Schedule and Section 100 (Highly Specialised Drug) listings be changed to Authority Required (STREAMLINED) and amendments to the General Statement for Drugs for the treatment of Hepatitis C to allow for this change.</p> <p>Sponsor Comment:</p>	<p>The PBAC deferred making a recommendation on amending the listing of grazoprevir with elbasvir from Authority Required (In Writing or Telephone) to Authority Required (STREAMLINED), without discussion. The PBAC considered that further consultation was required on the proposed changes to the HCV treatment matrix given the introduction of new Direct Acting Antivirals (DAA) treatments since the general statement was developed.</p> <p>MSD is committed to the pursuit of Hepatitis C elimination. We welcome further consultation on potential changes to the HCV treatment matrix to improve access for patients and retain the importance of genotyping to enable necessary individualised patient care.</p>

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<p>MEPOLIZUMAB</p> <p>Powder for injection 100 mg</p> <p>Nucala®</p> <p>GlaxoSmithKline Australia Pty Ltd</p> <p>Change to listing (Minor Submission)</p>	<p>Severe eosinophilic asthma</p>	<p>To request changes to the current Section 100 (Highly Specialised Drugs Program) Authority Required listing by removing the 6 month waiting period between the reuse of, or switching between omalizumab and mepolizumab.</p>	<p>The PBAC deferred making a decision on the request to remove the six month treatment break when switching between different biologics for the treatment of uncontrolled severe eosinophilic asthma and uncontrolled severe allergic asthma. The PBAC considered that removal of the treatment break would have flow-on implications and would require consideration of issues around re-trialling of the same biologic, switching between and cycling of biologics in severe asthma. The PBAC deferred its decision to enable further consideration and broader discussion given the complexity of these matters.</p>
		<p>Sponsor Comment:</p>	<p>The sponsor had no comment.</p>
<p>OBINUTUZUMAB</p> <p>Solution for I.V. infusion 1000 mg in 40 mL</p> <p>Gazyva®</p> <p>Roche Products Pty Ltd</p> <p>Change to listing (Minor Submission)</p>	<p>CD20 positive follicular lymphoma</p>	<p>Resubmission to request an Authority Required (STREAMLINED) listing for untreated patients with Stage II bulky or Stage III/IV CD20 positive follicular lymphoma or rituximab-refractory follicular lymphoma.</p>	<p>The PBAC deferred making a decision regarding the listing of obinutuzumab for previously untreated advanced follicular lymphoma (Stage II bulky or Stage III/IV) and rituximab-refractory follicular lymphoma to allow further work to establish a price for treatment with obinutuzumab that could be considered cost-effective in both these follicular lymphoma settings.</p> <p>The PBAC considered that for both treatment settings, the financial impact was uncertain. In the previously untreated setting, the PBAC considered that the resubmission had underestimated the amount of additional use of maintenance therapy. No financial estimates had been provided for the rituximab-refractory follicular lymphoma setting.</p> <p>The PBAC noted that resubmission proposed obinutuzumab be used in combination with bendamustine in the rituximab-refractory re-induction setting, but that bendamustine is not currently PBS-listed for such use. In recognising the clinical need for additional treatments in this setting, the PBAC indicated that it would be interested to consider listing bendamustine, for use in combination with obinutuzumab, in rituximab-refractory follicular lymphoma.</p> <p>The PBAC deferred its decision to allow these matters to be further clarified and explored.</p>
		<p>Sponsor Comment:</p>	<p>Roche is committed to working with the Department of Health and the PBAC to enable access to obinutuzumab for people with advanced follicular lymphoma.</p>

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<p>PEMBROLIZUMAB</p> <p>Powder for injection 50 mg Solution concentrate for I.V. infusion 100 mg in 4 mL</p> <p>Keytruda®</p> <p>Merck, Sharp & Dohme (Australia) Pty Ltd</p> <p>Change to listing (Minor Submission)</p>	<p>First-line treatment of patients with metastatic non-small cell lung cancer (NSCLC)</p>	<p>Resubmission to request a Section 100 (Efficient Funding of Chemotherapy) Authority Required (STREAMLINED) listing as first line monotherapy in patients expressing PD-L1 for NSCLC.</p>	<p>The PBAC deferred making a recommendation to list pembrolizumab for the first line treatment of patients with metastatic (Stage IV) non-small cell lung cancer (NSCLC), who have high expression of programmed cell death ligand 1 (PD-L1), defined as a tumour proportion score (TPS) of ≥50%. In deciding to defer, the PBAC advised that its primary concern was that further work was required to determine the size of the patient population eligible for treatment with pembrolizumab to inform the risk sharing arrangement. The PBAC also indicated its preference for a further price reduction to minimise the indirect, uncertain and delayed reliance on exceeding expenditure caps to achieve cost effectiveness. The PBAC considered that a further reduction in the cost per patient of pembrolizumab might also be required to address the residual uncertainties in the economic evaluation.</p>
		<p>Sponsor Comment:</p>	<p>MSD is disappointed that access to Keytruda has still not yet been achieved for 1L NSCLC patients in Australia. MSD will continue to work with the Department to make this therapy available as soon as possible, so that Australian patients have comparable access to innovative NSCLC therapies as patients in other countries.</p>

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<p>SAPROPTERIN</p> <p>Tablet (soluble) containing sapropterin dihydrochloride 100 mg</p> <p>Kuvan®</p> <p>BioMarin Pharmaceutical Australia Pty Ltd</p> <p>Change to listing (Major Submission)</p>	<p>Hyperphenylalaninaemia (HPA)</p>	<p>To request an Authority Required listing for the treatment of HPA in patients with phenylketonuria.</p>	<p>The PBAC deferred its decision about whether to recommend listing sapropterin for the treatment of patients with hyperphenylalaninaemia (HPA) caused by phenylketonuria (PKU). The PBAC sought further evidence regarding processes for determining whether or not a patient is responsive to sapropterin and the patient population in which treatment would result in the greatest benefit, in terms of clinically significant outcomes such as cognitive function and supporting growth. The PBAC considered the greatest benefits would be experienced in children and adolescents, and sought further advice regarding the age level of patients who would benefit most from sapropterin therapy. The PBAC noted this is a small patient population, and considered there were equity of access issues given that hospital-funded access was available in some states but not others.</p> <p>In deciding to defer, the PBAC acknowledged the input received from individuals, organisations and health professionals. The PBAC considered that the resulting incremental cost-effectiveness ratio (ICER) in patients aged 18 years and over, of >\$200,000 per quality-adjusted life year (QALY), was uncertain and unacceptably high. The PBAC noted that the ICERs for patients under 18 years of age were generally lower. The Committee considered that the clinically significant outcomes in younger patients may not have been fully captured in the economic evaluation.</p>
		<p>Sponsor Comment:</p>	<p>BioMarin would like to recognise the input received from patients, families and health professionals. BioMarin would also like to thank the PBAC for its consideration of PKU, a rare disease affecting about 1 in 15,000 newborns in Australia. BioMarin looks forward to continuing to work with the PBAC and Department of Health to improve access to innovative medicines for patients with PKU.</p>

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<p>SOFOSBUVIR WITH VELPATASVIR AND VOXILAPREVIR</p> <p>Tablet containing sofosbuvir 400 mg with velpatasvir 100 mg and voxilaprevir 100 mg</p> <p>Vosevi®</p> <p>Gilead Sciences Pty Ltd</p> <p>New listing (Major Submission)</p>	<p>Chronic hepatitis C virus (HCV) infection</p>	<p>To request a General Schedule and Section 100 (Highly Specialised Drugs Program) listing for the treatment of HCV infection in adults who have failed treatment with an NS5A-based direct acting antiviral treatment regimen, regardless of genotype.</p> <p>Sponsor Comment:</p>	<p>The PBAC deferred making a recommendation on the listing of sofosbuvir with velpatasvir and voxilaprevir without discussion, to allow further consideration of the issues raised within the sponsor's pre-PBAC response. The PBAC also considered that further consultation was required on the proposed changes to the HCV treatment matrix given the introduction of new Direct Acting Antivirals (DAA) treatments since the general statement was developed.</p> <p>The sponsor had no comment.</p>
<p>TENOFOVIR ALAFENAMIDE WITH EMTRICITABINE AND BICTEGRAVIR</p> <p>Tablet containing tenofovir alafenamide 25 mg with emtricitabine 200 mg and bicitgravir 50 mg</p> <p>Biktarvy®</p> <p>Gilead Sciences Pty Ltd</p> <p>New listing (Major Submission)</p>	<p>Human immunodeficiency virus (HIV) infection</p>	<p>To request a Section 100 (Highly Specialised Drugs Program - Community Access) Authority Required (STREAMLINED) listing for the treatment of patients with HIV infection.</p> <p>Sponsor Comment:</p>	<p>The PBAC was of a mind to recommend the Authority Required (STREAMLINED) Section 100 listing for a fixed dose combination (FDC) tablet containing tenofovir alafenamide with emtricitabine and bicitgravir (BFTAF) for the treatment of patients with human immunodeficiency virus (HIV-1), but deferred its decision pending the receipt of advice about the Therapeutic Goods Administration's (TGA) regulatory decision for this medicine. The PBAC considered that the cost-effectiveness of BFTAF would be acceptable if it were cost-minimised against the lowest priced alternative FDC triple-therapy for the treatment of HIV (i.e. tenofovir disoproxil-based (TDF) alternatives to the nominated secondary comparators GENVOYA® and ODEFSEY®, STRIBILD® and EVIPLERA®).</p> <p>Gilead notes that the PBAC accepted that the nominated comparators of DESCOVY® plus dolutegravir, GENVOYA® and ODEFSEY® were appropriate. These are the regimens that it is agreed are most likely to be replaced in clinical practice. Older fixed dose regimens have been superseded by newer, safer treatments and no longer represent alternative therapies.</p>

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<p>TOLVAPTAN</p> <p>Pack containing 28 tablets 15 mg and 28 tablets 45 mg Pack containing 28 tablets 30 mg and 28 tablets 60 mg Pack containing 28 tablets 30 mg and 28 tablets 90 mg</p> <p>Jinarc®</p> <p>Otsuka Australia Pharmaceutical Pty Ltd</p> <p>New listing (Major Submission)</p>	<p>Autosomal dominant polycystic kidney disease (ADPKD)</p>	<p>Resubmission to request an Authority Required listing for the treatment of ADPKD.</p>	<p>The PBAC deferred making a recommendation on whether tolvaptan should be listed on the PBS for the treatment of autosomal dominant polycystic kidney disease (ADPKD) to allow further discussion and modelling to establish a price that could be considered cost effective. The PBAC accepted the high clinical need for effective therapy to treat ADPKD, however it was considered based on the evidence provided that the clinical benefit of tolvaptan treatment was uncertain and at best very small. The PBAC noted that no new trial data is anticipated that could resolve this issue and that treatment is also expected to be long-term; over 40-50 years. In this context the PBAC considered a further submission for PBS listing would need to resolve the issues of uncertain clinical benefit and duration of therapy by adjusting the optimistic assumptions in the economic evaluation. The price would also need to be substantially lower to reduce the incremental cost effectiveness ratio (ICER). Further work on the proposed PBS restriction would also be required, noting the inclusion of rapid disease progression was problematic.</p>
		<p>Sponsor Comment:</p>	<p>Otsuka is disappointed that a recommendation on tolvaptan has been deferred. Tolvaptan is already available as a first in class treatment for ADPKD in Japan, Canada, Europe and other countries. There are no other treatments available for this small patient population in Australia. Otsuka is committed to working with the PBAC and the Department to find a way of making tolvaptan accessible to patients as soon as possible.</p>