PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
SEPTEMBER 2021 PBAC MEETING

The PBAC outcomes and recommendations are presented in alphabetical order by drug name.

Submission items

DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION	DRUG TYPE AND USE	LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION	PBAC OUTCOME	
DAPAGLIFLOZIN Tablet 10 mg Forxiga® AstraZeneca Pty Ltd Matters Outstanding (Change to PBS listing)	Chronic kidney disease (CKD)	To request a General Schedule, Authority Required (STREAMLINED) listing for the treatment of CKD.	Not Recommended	The PBAC did not recommended extending the existing listing of dapagliflozin to include a General Schedule Authority Required (STREAMLINED) listing for the treatment of patients with CKD. Following a deferred decision in July 2021, the PBAC reiterated that it was satisfied that dapagliflozin added to standard care provides, for some patients, a significant improvement in efficacy over standard care alone. The PBAC remained of the view that the listing would be cost-effective at the price proposed in the pre-PBAC response from July 2021. In July 2021, the PBAC had been concerned that the financial estimates were high and uncertain, that the eligible population had been very significantly overestimated, and there was a risk of use outside the proposed restriction. The PBAC had made recommendations in terms of revisions required to the assumptions informing the financial estimates for CKD. The PBAC had requested additional information to estimate the overall net impact of listing for both heart failure with reduced ejection fraction and CKD, in addition to use for type 2 diabetes mellitus to inform its advice about an appropriate Risk Sharing Arrangement (RSA). The Department obtained additional information from the sponsor, but the PBAC remained of the view that the financial estimates for CKD remained high and substantially overestimated (in terms of eligible population size), and as such did not form a reliable basis for an RSA.
				Sponsor's Comment: The sponsor had no comment.
DAPAGLIFLOZIN Tablet 10 mg Forxiga®	Heart failure	To request a General Schedule, Authority Required (STREAMLINED) listing for the treatment of heart failure with reduced ejection fraction (HFrEF).	Recommended	The PBAC recommended extending the existing listing of dapagliflozin to include a General Schedule Authority Required (STREAMLINED) listing for the treatment of patients with HFrEF. Following a deferred decision in July 2021, the PBAC reiterated that it was satisfied that dapagliflozin added to standard care provides, for some patients, a significant improvement in efficacy over

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AstraZeneca Pty Ltd Matters Outstanding (Change to PBS listing)				standard care alone. The PBAC remained of the view that the listing would be cost-effective at the price proposed in the pre-PBAC response from July 2021. In July 2021, the PBAC had been concerned about the risk of use outside the proposed PBS restriction. The PBAC had requested additional information to estimate the overall net impact of listing for both HFrEF and chronic kidney disease, in addition to use for type 2 diabetes mellitus to inform its advice about an appropriate Risk Sharing Arrangement (RSA). The Department obtained additional information from the sponsor, and the PBAC considered that the estimates provided for HFrEF were broadly reasonable, and would be an appropriate basis for an RSA for this indication (with minor reductions to the expected uptake in Year 1).
METHOXSALEN Solution for blood fraction 20 microgram per mL, 10 mL Uvadex® Terumo BCT Australia Pty Ltd Matters Outstanding (Change to PBS listing)	Steroid dependent or steroid intolerant or steroid refractory chronic graft versus host disease (cGVHD)	To request a Section 100 (Highly Specialised Drugs Program) Authority Required (STREAMLINED) listing for the treatment of patients with steroid dependent or steroid intolerant or steroid refractory cGVHD, as part of treatment with integrated, closed system extracorporeal photopheresis (ECP).	Recommended	The PBAC recommended the Section 100 (Highly Specialised Drugs Program – Public and Private Hospital) Authority Required (STREAMLINED) listing for methoxsalen, delivered as part of an integrated, closed system ECP service for the treatment of patients with steroid dependent, steroid intolerant or steroid refractory cGVHD. The PBAC was satisfied that ECP involving methoxsalen provides, for some patients, a significant improvement in efficacy over current standard of care. The PBAC noted that MSAC accepted that ECP had likely superior clinical effectiveness and non-inferior safety compared with current standard of care. Further, the PBAC noted that MSAC advised that ECP plus methoxsalen has acceptable cost-effectiveness in the treatment of cGVHD compared with current standard of care for the proposed patient population.
NINTEDANIB Capsule 100 mg Capsule 150 mg Ofev® Boehringer Ingelheim Pty Ltd Early Resolution Resubmission (Change to PBS listing)	Progressive fibrosing interstitial lung disease (PF-ILD)	Resubmission to request an Authority Required (Written) listing for the treatment of PF-ILD.	Recommended	The PBAC recommended the listing of nintedanib for the treatment of patients with PF-ILD. The PBAC was satisfied that nintedanib provides, for some patients, a significant improvement in effectiveness compared with best supportive care. The PBAC considered that the remaining uncertainty around the cost-effectiveness of nintedanib could be adequately managed by a price reduction. The PBAC was satisfied that the proposed Risk Sharing Arrangement would manage the financial risk of utilisation outside the eligible patient population. The PBAC also considered that the revised estimated utilisation and financial estimates were reasonable.

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ONASEMNOGENE ABEPARVOVEC Solution for injection, customised based on patient weight Zolgensma® Novartis Pharmaceuticals Australia Pty Ltd Matters Outstanding (New PBS listing)	Spinal muscular atrophy (SMA)	To request an Authority Required (Written) listing for the treatment of paediatric patients with Type 1 SMA.	Recommended	The PBAC recommended the Section 100 (Highly Specialised Drugs Program) listing of onasemnogene abeparvovec (ONA) for the treatment of SMA in patients aged less than 9 months, with Type 1 SMA or pre-symptomatic patients with 1-2 copies of the SMN2 gene. The PBAC's recommendation for listing was based on, among other matters, its assessment that the cost-effectiveness of ONA would be acceptable if it were cost-minimised to the least costly disease-modifying therapy for this condition, which is risdiplam. The PBAC also considered that an outcomes-based Risk Sharing Arrangement would be required to mitigate the financial risk to the Commonwealth associated with upfront payment for ONA where the treatment fails, resulting in death, permanent ventilation or lack of efficacy.
TAFAMIDIS Capsule 61 mg Vyndamax® Pfizer Australia Pty Ltd Early Resolution Resubmission (New PBS listing)	Transthyretin amyloid cardiomyopathy (ATTR-CM)	Resubmission to request an Authority Required listing for the treatment of ATTR-CM.	Not Recommended	The PBAC did not recommend the listing of tafamidis for the treatment of ATTR-CM. The PBAC considered that its previous concerns were not adequately addressed. In particular, the PBAC considered that the incremental cost-effectiveness ratio (ICER) stated in the resubmission was significantly higher than the ICER requested. Further, the PBAC considered that the proposed Risk Sharing Arrangement, with expenditure caps higher than the resubmission's estimated financial expenditure, would not adequately manage the risk associated with use in a broader population in which cost-effectiveness is unknown. The resubmission was lodged under the Early Resolution resubmission pathway. The comparator, clinical claim and economic claim were unchanged from the previous submission.
				Sponsor's Comment: Pfizer is disappointed that the substantial changes proposed were not adequate to secure a positive recommendation from the PBAC to make tafamidis available for Australian patients with ATTR-CM.

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Non-submission items

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CHORIOGONADOTROPIN ALFA Solution for injection 250 micrograms in 0.5 mL pre-filled pen Ovidrel® Merck Healthcare Pty Ltd	In-vitro fertilisation and infertility	The PBAC to consider a new, Section 85 listing for choriogonadotropin alfa, in addition to its Section 100 listing, following discussion of the discontinuation of human chorionic gonadotrophin (Pregnyl®) at the July 2021 PBAC Meeting.	The PBAC recommended the General Schedule listing of choriogonadotropin alfa solution for injection 250 micrograms in 0.5 mL pre-filled pen (Ovidrel) for the treatment of infertility (not associated with assisted reproductive technology).

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DOXORUBICIN (AS PEGYLATED LIPOSOMAL) Suspension for I.V. infusion containing pegylated liposomal doxorubicin hydrochloride 20 mg in 10 mL Suspension for I.V. infusion containing pegylated liposomal doxorubicin hydrochloride 50 mg in 25 mL Caelyx® Liposomal Doxorubicin Sun® Janssen-Cilag Pty Ltd Sun Pharma ANZ Pty Ltd RALTITREXED Powder for I.V. infusion 2 mg in single use vial Tomudex® Pfizer Australia Pty Ltd	Multiple indications	The PBAC to consider a Medical Oncology Group of Australia submission seeking the complete derestriction of raltitrexed, and to also consider a potential unrestricted listing for liposomal doxorubicin.	The PBAC recommended that the PBS listings for doxorubicin (as pegylated liposomal) and raltitrexed be changed to Unrestricted Benefit listings. For doxorubicin, the PBAC considered that a potential benefit of an unrestricted listing would be improved access for patients with rare sarcomas, endometrial cancer, and frail patients unable to tolerate standard treatment. For raltitrexed, the PBAC considered that an unrestricted listing would enable more effective ongoing combination chemotherapy if there is fluorouracil cardiotoxicity. The PBAC considered there would be acceptable cost-effectiveness in the broader populations and minimal financial impact.

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RITUXIMAB Solution for I.V. infusion 100 mg in 10 mL Solution for I.V. infusion 500 mg in 50 mL Riximyo® Truxima® Sandoz Pty Ltd Cellitron Healthcare Australia Pty Ltd	Multiple indications	The PBAC to consider a number of rituximab issues at the same time. This includes the impact on the existing biosimilar brands (Riximyo and Truxima) and biosimilar uptake drivers due to the delisting of the reference brand of rituximab IV (MabThera®). Other issues include the potential extension of the rituximab PBS listings following numerous enquiries/requests in previous years.	The PBAC recommended removal of the administrative notes related to the biosimilar uptake drivers for rituximab, in light of the removal of the reference brand of rituximab, MabThera, from the PBS. The PBAC recommended that the PBS listings for all listed brands of rituximab be changed to Unrestricted Benefit listings. The PBAC considered that changing the rituximab listings to unrestricted would provide subsidised access to treatment for patients with conditions where there are no alternative PBS listed medicines. The PBAC considered the expanded use would be cost effective given the price reductions to rituximab to date and scheduled from 1 October 2021. The PBAC noted the increased projected utilisation and requested that utilisation be reviewed after 18 months of the unrestricted listing.
ROMOSOZUMAB Injection 105 mg in 1.17 mL single use pre-filled syringe Evenity® Amgen Australia Pty Ltd TERIPARATIDE Injection 250 micrograms per mL, 2.4 mL in multi-dose pre-filled pen Forteo® Eli Lilly Australia Pty Ltd	Osteoporosis	The PBAC to consider correspondence from Healthy Bones Australia requesting that the romosozumab listing be corrected so that previous non-PBS patients can now be eligible to receive subsidy for these medicines.	The PBAC recommended the addition of the words ‘PBS-subsidised treatment’ to the romosozumab and teriparatide restrictions to ensure that patients who previously self-funded their romosozumab or teriparatide treatment are not excluded. This reflects the intent of the Committee’s recommendation for romosozumab at the March 2020 PBAC meeting.

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SOMATROPIN Multiple forms and strengths Multiple brands and sponsors	Severe Growth Hormone Deficiency	The PBAC to consider requests from the Australasian Paediatric Endocrine Group (APEG) to change the somatropin eligibility criteria.	The PBAC accepted several requested changes to the restrictions for somatropin from the APEG. The PBAC noted that the proposed restriction changes would have a high clinical benefit. The PBAC considered that the estimated financial impact was acceptable.
TOCILIZUMAB Concentrate for injection 80 mg in 4 mL Concentrate for injection 200 mg in 10 mL Concentrate for injection 400 mg in 20 mL Injection 162 mg in 0.9 mL single use pre-filled syringe Actemra® Roche Products Pty Ltd	Multiple indications	The PBAC to consider the suggested changes from the Australian Rheumatology Association regarding the management of switching between bDMARDs during the tocilizumab shortage.	The PBAC recommended the implementation of new temporary listings under General Schedule and Section 100 (Highly Specialised Drugs) to facilitate the switch to other bDMARDs and return to tocilizumab therapy during the tocilizumab shortage. The PBAC was supportive of extending the requirement of a demonstrated response from 16 to 24 weeks. The PBAC advised these temporary arrangements should be in place for the duration of the tocilizumab shortage, with the arrangements ceasing on resolution of the shortage.

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Resubmission pathways

*There are four different resubmission pathways available to applicants following a 'not recommended' PBAC outcome. Resubmission pathways are not available for submissions that receive a positive recommendation from the PBAC. The resubmission pathways are classified into the following categories:	
Standard re-entry	The Standard Re-entry Pathway is the default pathway for resubmissions and also applies where: • an applicant chooses not to accept the PBAC nominated resubmission pathway; or • an Early Re-entry or Early Resolution Pathway has been nominated by the PBAC and an applicant decides to address issues other than those identified by the PBAC (including a subset of issues); or • an applicant decides to lodge later than the allowable timelines for the other pathways.
Early re-entry pathway	An Early Re-entry Pathway may be nominated by the PBAC where the PBAC considers that the remaining issues could be easily resolved and the medicine or vaccine does not represent HATV for the proposed population. Applicants who accept this pathway are eligible for PBAC consideration at the immediate next meeting.
Early resolution pathway	For medicines or vaccines deemed by the PBAC to represent High Added Therapeutic Value (HATV) AND where the PBAC considers that the remaining issues could be easily resolved, including when: • new clinical study data requiring evaluation is not considered necessary by the PBAC to support new clinical claims to be made in the resubmission; and • a revised model structure or input variable changes (beyond those specified by the PBAC) are not necessary to support any new economic claims, or to estimate the utilisation and financial impacts to be made in the resubmission. Applicants who accept this pathway are eligible for PBAC consideration out-of-session (before the main meeting), unless the department, in consultation with the PBAC Chair, identifies an unexpected issue such that the resubmission needs consideration at the next main PBAC meeting.
Facilitated resolution pathway	A Facilitated Resolution Pathway may be nominated by the PBAC where the PBAC considers the issues for resolution could be explored through a workshop AND where the medicine or vaccine meets the HATV criteria. Applicants who accept this pathway are eligible for a solution-focussed workshop with one or more members of the PBAC. The workshop agenda will be based on the issues for resolution outlined in the PBAC Minutes. This can be further clarified during the post-PBAC meeting with the Chair.