**PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES**
**DECEMBER 2023 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 | |
|---|---|---|---|---|
| PATISIRAN<br><br>Solution concentrate for I.V. infusion 10 mg in 5 mL<br><br>Onpattro®<br><br>Alnylam Australia Pty Ltd<br><br>(New PBS listing) | Hereditary transthyretin-mediated amyloidosis (hATTR amyloidosis) | To consider a resubmission requesting the listing of patisiran for the treatment of hATTR amyloidosis with polyneuropathy. | Recommended | The PBAC recommended patisiran for the treatment of patients with hATTR amyloidosis with polyneuropathy. The PBAC noted there was a high clinical need for effective treatment in this population. The PBAC noted the clinical benefits of patisiran in terms of delaying disease progression, reducing neuropathy symptoms, and improving health-related quality of life compared to placebo, based on the primary and secondary outcomes in the APOLLO trial over its 18-month trial period.<br><br>The PBAC recalled that in September 2023 it had advised that revisions were required to the economic model and that concerns remained regarding uncertainty of the cost-effectiveness estimates and the financial estimates. The PBAC considered that the outstanding issues regarding the economic model had been satisfactorily resolved by the revised proposal provided by the sponsor, which included a commitment for reassessment of the data after three years. The PBAC advised that further amendments to the financial estimates were necessary to reflect the anticipated discontinuation rate. |
| UPADACITINIB<br><br>Tablet 15 mg<br><br>Rinvoq®<br><br>AbbVie Pty Ltd<br><br>(Change to listing) | Severe active rheumatoid arthritis (RA) | A resubmission to request a change to the restriction level of upadacitinib in the subsequent continuing treatment of RA from Authority Required (Written) to Authority Required (STREAMLINED). | Recommended | The PBAC recommended an amendment to the written authority levels of upadacitinib for the treatment of severe active RA to Authority Required (Written) for first continuing treatment and Authority Required (STREAMLINED) for subsequent continuing treatment. The PBAC noted the market of upadacitinib was mature and upadacitinib initiations had stabilised. It therefore considered that upadacitinib would not drive growth in the RA market. The PBAC considered it was appropriate to align the restriction levels of upadacitinib with its March 2022 PBAC meeting recommendation for RA medicines. |

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*Non-submission items*

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| ALECTINIB, BRIGATINIB, CERITINIB, CRIZOTINIB, ENTRECTINIB, AND LORLATINIB<br><br>All forms and strengths<br><br>Various brands<br><br>Various sponsors<br><br>(Change to listing) | Non-small cell lung cancer (NSCLC) | To consider a proposed change to the restriction criteria to amend testing requirements for identification of anaplastic lymphoma kinase (ALK) or c-ROS proto-oncogene 1 (ROS1) gene rearrangement in tumour material to include next generation sequencing gene panel testing for NSCLC as an eligible testing method for PBS subsidy in addition to fluorescence in situ hybridisation (FISH) testing, following the 1 November 2023 listing of next generation sequencing (NGS) gene panel testing for NSCLC on the Medicare Benefits Schedule. | Recommended | The PBAC recommended changes to the restriction criteria for alectinib, brigatinib, ceritinib, crizotinib, entrectinib and lorlatinib (medicines that require evidence of the presence of ALK or ROS1 gene rearrangement), to include small NGS gene panel biomarker testing as an eligible testing method for PBS subsidy in addition to FISH biomarker testing. |

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| ASCIMINIB, DASATINIB, IMATINIB, NILOTINIB, AND PONATINIB<br><br>All forms and strengths<br><br>Various brands<br><br>Various sponsors<br><br>(Change to listing) | Chronic myeloid leukaemia (CML) | To consider updates to the restrictions of tyrosine kinase inhibitors (TKIs) for the treatment of CML to align with current clinical practice. | Recommended | The PBAC recommended the following changes to the PBS restriction criteria for the imatinib, dasatinib and nilotinib listings for CML:<br>• remove specification of line of treatment and phase<br>• allow all strengths of nilotinib to be available for dose titration regardless of line of therapy.<br><br>The PBAC recommended removing 'accelerated phase' from the PBS restriction criteria for all five TKIs to align with the latest World Health Organization (WHO) classification. The PBAC advised that 'not blast phase (BP)' continue to be included in the restriction criteria for asciminib, and that prescribers should be referred to the WHO website for the current definition of BP. The PBAC recommended the following wording be removed from the ponatinib PBS restriction criteria: 'Patient must not be eligible for PBS-subsidised treatment with nilotinib because the patient has a blast crisis.' The PBAC recommended that the prescribing/administrative advice around diagnosis, definition of response, and definition of nonresponse be removed from the PBS restriction criteria, as a select group of clinicians prescribe these targeted therapies for CML (i.e., specialist haematologists). The PBAC considered these changes would not result in any material changes to the patient population eligible for TKIs listed on the PBS for CML and therefore would not result in increased costs to the PBS. |
| MELATONIN<br><br>Tablet 1 mg<br>Tablet 5 mg<br><br>Slenyto®<br><br>Aspen Pharmacare Australia Pty Ltd<br><br>(Matters arising) | Insomnia in patients between the ages of 2 to 18 with Smith-Magenis syndrome (SMS) | To consider whether to amend, rescind or extend the PBAC's previous recommendation in November 2021 to list Slentyo 1 mg and 5 mg tablets on the PBS for the treatment of insomnia in patients with SMS. | Recommended | The PBAC amended the November 2021 recommended listing of melatonin for the treatment of insomnia in patients with SMS. The PBAC considered that the requirement for a Risk Sharing Arrangement could be removed if the restriction included a criterion that required patients to undergo genetic testing to confirm diagnosis of SMS. |

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| NIVOLUMAB plus IPILIMUMAB<br><br>Nivolumab:<br>Injection concentrate for I.V. infusion 40 mg in 4 mL<br>Injection concentrate for I.V. infusion 100 mg in 10 mL<br><br>Opdivo®<br><br>Ipilimumab:<br>Injection concentrate for I.V. infusion 50 mg in 10 mL<br>Injection concentrate for I.V. infusion 200 mg in 40 mL<br><br>Yervoy®<br><br>Bristol-Myers Squibb Australia Pty Ltd<br><br>(Change to listing) | Multiple indications | To consider a proposal for an expanded listing to facilitate broad access to indications with current or future Therapeutic Goods Administration (TGA) registration. | Deferred | The PBAC deferred making a recommendation for an expanded listing of nivolumab and ipilimumab, which would encompass all indications with current or future TGA registration. The PBAC considered, in the context of the extensive experience with applications for PD-(L)1 inhibitors, that it would be appropriate and desirable to have a simplified process for listing future indications. The PBAC considered a broad listing for nivolumab would be appropriate with a Risk Sharing Arrangement (RSA) that provided confidence regarding total cost to Government and cost-effectiveness of a broad listing, and that a substantial price reduction versus the current PBS prices would likely be required. The PBAC considered further consultation was required with the sponsor and the Department regarding the restriction, the price at which nivolumab is likely to be cost-effective, the financial forecasts and parameters for the RSA.<br><br><u>Sponsor's comment:</u><br><br>Bristol-Myers Squibb Australia (BMSA) appreciates the opportunity to partner with the PBAC in seeking innovative ways for patients to gain early and broad access to life saving medicines. BMSA are committed to working with the PBAC to explore the potential for an expanded listing for nivolumab +/- ipilimumab in line with current and future TGA registrations and are hopeful that additional consultation may lead to such access. |

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| PEMBROLIZUMAB<br><br>Solution concentrate for I.V. infusion 100 mg in 4 mL<br><br>Keytruda®<br><br>Merck Sharp & Dohme (Australia) Pty Ltd<br><br>(Change to listing) | Multiple indications | To consider a proposal for an expanded listing to facilitate broad access to indications with current or future TGA registration. | Deferred | The PBAC deferred making a recommendation for an expanded listing of pembrolizumab which would encompass all indications with current or future TGA registration. The PBAC considered, in the context of the extensive experience with applications for PD-(L)1 inhibitors, that it would be appropriate and desirable to have a simplified process for listing future indications. The PBAC considered a broad listing for pembrolizumab would be appropriate with an RSA that provided confidence regarding total cost to Government, and cost-effectiveness of a broad listing, and that a substantial price reduction versus the current PBS prices would likely be required. The PBAC considered further consultation was required with the sponsor and the Department regarding the restriction, the price at which pembrolizumab is likely to be cost-effective, the financial forecasts and parameters for the RSA.<br><br><u>Sponsor's comment:</u><br><br>Merck Sharp & Dohme (MSD) looks forward to working with the PBAC and the Department of Health and Aged Care to resolve the outstanding issues so that a broad PBS listing for Keytruda can become a reality for Australian cancer patients as soon as possible. |
| Review of PBS prescribing for nurse practitioners (NP) and endorsed midwives (EM) on the PBS<br><br>(PBS review) | Various | To provide the PBAC with an update on the progress of the review. | Advice provided | The PBAC noted that a review is being undertaken by the Department on PBS-listed medicines that may be appropriate for NP or EM prescribing. The PBAC noted the Department's intent to undertake an open consultation on the review of NP and EM prescribing in early 2024. The findings of the consultation and a consolidated list of medicines identified as priorities for NP and EM prescribing on the PBS are to be considered by the PBAC at a subsequent meeting. The PBAC advised that supportive reasons for each identified medicine, specifically addressing prescriber competency and clinical settings in which prescribing would occur, would assist the Committee in recommending any changes to the eligible prescriber types and/or the circumstances of listing of the identified medicines.<br><br>The PBAC and Department plan to consult with health professional groups in the development of general principles for determining prescriber types for PBS listings. The PBAC indicated that it would give further consideration to the guidance principles for determining PBS prescriber eligibility at a future meeting. |

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| Update on the revised Post-market Review (PMR) Framework<br><br>(PBS Review) | Various indications | To update the PBAC on the revised PMR Framework prior to Ministerial approval. | Endorsed | The PBAC recalled that in 2022, the 2015 PMR Framework was reconsidered in the context of the new Strategic Agreement between the Commonwealth and Medicines Australia (MA).<br><br>The PBAC considered the key changes between the 2015 PMR Framework and the 2024 PMR Framework which included: improved transparency of new PMR topics and the status of ongoing Reviews through periodic publication of a PMR workplan; standardised Review Terms of Reference (ToRs); and the formation of a Review Reference Group only when requested by the PBAC.<br><br>The PBAC noted that under the 2024 PMR Framework the PBAC Executive would approve the final Review ToRs in most cases unless there was the need for a significant departure from the standardised ToRs; as opposed to the 2015 PMR Framework in which the final ToRs are approved by the full Committee and the Minister.<br><br>The PBAC noted that the 2024 PMR Framework was developed in consultation with MA and considered feedback from stakeholders received as part of a public consultation process from 20 October 2022 to 16 December 2022.<br><br>The PBAC endorsed the changes made in the 2024 PMR Framework and noted that the revised Framework would be published on the PBS website following Ministerial approval in early 2024. The PBAC noted that following approval of the revised process by the Minister, and not limiting the Committee's independence, all Committee requests for a PMR of PBS-listed medicines would follow the process outlined in the 2024 PMR Framework. |

Version 2
1. Update on the revised Post-market Review (PMR) Framework – web outcome added.

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**Resubmission pathways**

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| *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:<br>• an applicant chooses not to accept the PBAC nominated resubmission pathway; or<br>• 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<br>• 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 High Added Therapeutic Value (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 HATV AND where the PBAC considers that the remaining issues could be easily resolved, including when:<br>• 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<br>• 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.<br>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. |