**PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES**
**MARCH 2022 PBAC MEETING**

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

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| ABEMACICLIB<br><br>Tablet 50 mg<br>Tablet 100 mg<br>Tablet 150 mg<br><br>Verzenio™<br><br>Eli Lilly Australia Pty Ltd<br><br>Category 2 submission<br>(Change to listing) | Breast cancer | To request a General Schedule Authority Required listing, in combination with adjuvant endocrine therapy, for the treatment of patients with hormone receptor positive (HR+) and human epidermal growth factor receptor 2 negative (HER2-), lymph node-positive, invasive, resected early breast cancer and whose cancer is at high risk of disease recurrence. | Not Recommended | The PBAC did not recommend the listing of abemaciclib in combination with endocrine therapy for the treatment of patients with HR+ and HER2-, lymph node positive, invasive, resected, early breast cancer at high risk of recurrence. The PBAC considered there is high uncertainty regarding the extent of clinical benefit of abemaciclib due to low patient numbers with events and small absolute risk reduction. However, the PBAC considered that the claim of superior effectiveness was supported in the adjuvant setting where the goal is cure, notwithstanding that the claim of invasive disease-free survival being employed as a surrogate for overall survival was uncertain in the context of the data currently available. The PBAC considered that the inferior but manageable safety claim was reasonable, noting that abemaciclib is an existing therapy with a known safety profile and that adverse events could be monitored and managed with dose modifications.<br><br>The PBAC considered that the economic analysis was highly uncertain and that the economic model was not reliable for decision making. The PBAC also considered the assumptions and parameters were not reasonable, particularly time horizon, duration of treatment effect, and modelled implications of avoiding recurrence.<br><br>The PBAC considered that the financial estimates presented in the submission were overestimated and that a reduction to the assumed uptake and compliance rates and duration of therapy should be considered, as well as a review of the costs used for metastatic recurrence and adverse events. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| | | | | Sponsor Comment:<br>Eli Lilly is disappointed with the outcome given the genuine unmet need for a new treatment option in the adjuvant early breast cancer setting.<br>Abemaciclib for the adjuvant treatment of adult patients with early breast cancer at high risk of recurrence was scored the highest grade 'A' on the ESMO-MCBS scale, categorising abemaciclib as a treatment with substantial benefit in the curative setting, and was considered by MOGA to be a therapy of highest priority for PBS listing.<br>Eli Lilly is committed to work towards a PBS listing for patients with high-risk early breast cancer. |
| ABIRATERONE with METHYLPREDNISOLONE<br><br>Pack containing 120 tablets abiraterone (as acetate) 125 mg and 60 tablets methylprednisolone 4 mg<br><br>Yonsa® MPRED<br><br>Sun Pharma ANZ Pty Ltd<br><br>Category 2 submission<br>(New PBS listing) | Castration resistant metastatic carcinoma of the prostate | To request a General Schedule Authority Required (STREAMLINED) listing of a composite pack for the treatment of castration resistant metastatic carcinoma of the prostate. | Not Recommended | The PBAC did not recommend the listing of a composite pack (co-pack) comprising abiraterone acetate tablets in a fine particle formulation (SAA) and oral methylprednisolone (MPRED) tablets for the treatment of patients with metastatic castration resistant prostate cancer (mCRPC). The PBAC raised concerns regarding the quality use of the co-pack in practice, noting there would be a risk of confusion among patients due to differences in dosing of SAA and MPRED compared to the currently listed form of abiraterone and prednisolone. The PBAC considered further information from the sponsor would be required to clarify how these risks would be mitigated.<br><br>Sponsor Comment:<br>SUN is committed to working with the PBAC to make SAA+MPRED available to patients with mCRPC. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| AMIFAMPRIDINE<br><br>Tablet 10 mg<br><br>Ruzurgi®<br><br>The Trustee For Orspec Pharma Unit Trust<br><br>Early re-entry submission<br>(New PBS Listing) | Lambert-Eaton myasthenic syndrome | Resubmission to request a General Schedule Authority Required listing for the treatment of Lambert-Eaton myasthenic syndrome in adults and children aged six years and above. | Recommended | The PBAC recommended the listing of amifampridine for the treatment of Lambert-Eaton myasthenic syndrome (LEMS). The resubmission provided a revised economic model and financial estimates in response to previous concerns raised by the PBAC at the November 2021 meeting. The PBAC considered that the incremental cost-effectiveness ratio was acceptable in the context of LEMS being a rare condition with an unmet need for effective treatment. The PBAC noted that the financial estimates had been recalculated, consistent with previous advice. |
| APREMILAST<br><br>Tablet 30 mg<br>Pack containing 4 tablets 10 mg,<br>4 tablets 20 mg and 19 tablets 30 mg<br><br>Otezla®<br><br>Amgen Australia Pty Limited<br><br>Category 3 submission<br>(Change to PBS listing) | Severe chronic plaque psoriasis | To request changing the treatment criteria to allow accredited dermatology registrars to initiate treatment in consultation with a dermatologist; and to allow general practitioners to prescribe maintenance treatment. | Recommended | For further details please see the outcomes of the May 2022 PBAC meeting. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| AVELUMAB<br><br>Solution concentrate for I.V. infusion 200 mg in 10 mL<br><br>Bavencio®<br><br>Merck Healthcare Pty Ltd<br><br>Matters Arising<br>(Change to PBS listing) | Urothelial carcinoma | To request that the PBAC review a revised proposal following the March 2021 recommendation for avelumab for the first-line maintenance treatment of locally advanced (Stage III) or metastatic (Stage IV) urothelial carcinoma. | Advice Provided | The PBAC provided further advice in regard to its March 2021 recommendation for the listing of avelumab, for the maintenance treatment of locally advanced or metastatic urothelial carcinoma, and the sponsor's subsequent listing proposal which included modifications to the economic model. The PBAC advised that avelumab would be cost-effective at the price proposed by the sponsor together with a risk sharing arrangement. |
| BECLOMETASONE WITH FORMOTEROL<br><br>Pressurised inhalation containing beclometasone dipropionate 100 micrograms and formoterol fumarate dihydrate 6 micrograms per dose, 120 dose<br><br>Fostair®<br><br>Chiesi Australia Pty Ltd<br><br>Category 2 submission<br>(Change to PBS listing) | Chronic obstructive pulmonary disease (COPD) | To request a General Schedule Authority Required (STREAMLINED) listing for the symptomatic treatment of adults with severe COPD (FEV1 <50% predicted normal) and a history of repeated exacerbations, who have significant symptoms despite regular therapy with long-acting bronchodilators. | Recommended | The PBAC recommended the Authority Required (STREAMLINED) listing of the fixed-dose combination (FDC) of beclomethasone (BEC) with formoterol (FOR) for the treatment of COPD. The PBAC considered the claim of non-inferior effectiveness and safety to the FDC of fluticasone propionate (FP) 250 µg with salmeterol (SAL) 25 µg was reasonable. However, the PBAC considered for purposes of satisfying Section 101(3B) of the *National Health Act 1953*, any high dose inhaled corticosteroid (ICS) with long-acting beta2-agonist (LABA) FDC are relevant alternative therapies. The PBAC's recommendation was therefore, among other matters, based on its assessment that the cost of BEC/FOR should be no greater than the lowest price combination of the PBS listed components of ICS/LABA therapy that are available for COPD at comparable doses. Flow-on restriction changes to the administrative advice of the current BEC/FOR listing for asthma were also recommended. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| BECLOMETASONE WITH FORMOTEROL AND GLYCOPYRRONIUM<br><br>Pressurised inhalation containing beclometasone dipropionate 100 micrograms with formoterol fumarate dihydrate 6 micrograms and glycopyrronium 10 micrograms (as bromide) per dose, 120 doses<br><br>Pressurised inhalation containing beclometasone dipropionate 200 micrograms with formoterol fumarate dihydrate 6 micrograms and glycopyrronium 10 micrograms (as bromide) per dose, 120 doses<br><br>Trimbow®<br><br>Chiesi Australia Pty Ltd<br><br>Category 2 submission (New PBS listing) | Asthma | To request a General Schedule Authority Required (STREAMLINED) listing for the maintenance treatment of asthma in adult patients not adequately controlled with a maintenance combination of a long-acting beta2-agonist and an inhaled corticosteroid who experienced one or more asthma exacerbations in the previous year. | Deferred | The PBAC deferred making a recommendation for the Authority Required listing for a fixed dose combination (FDC) of beclometasone (BEC) with formoterol (FOR) and glycopyrronium (GLY) for the maintenance therapy of severe asthma as the TGA Delegate's Overview was not available at the time of consideration. However, the PBAC was of a mind to recommend listing BEC/FOR/GLY for this indication based on its assessment that the cost of BEC/FOR/GLY should be no greater than the lowest price combination of the PBS listed components of triple therapy that are available for asthma at comparable doses.<br><br><u>Sponsor Comment:</u><br>The sponsor had no comment. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| BEVACIZUMAB<br><br>Solution for I.V. infusion 100 mg in 4 mL<br>Solution for I.V. infusion 400 mg in 16 mL<br><br>Bevaciptin®<br><br>Cipla Australia Pty Ltd<br><br>Category 3 submission<br>(New PBS listing) | Cancers | To request a Section 100 (Efficient Funding of Chemotherapy Program) Unrestricted Benefit listing of bevacizumab biosimilar under the same conditions as the PBS-listed bevacizumab biosimilar. | Recommended | The PBAC recommended listing a new biosimilar brand of bevacizumab, Bevaciptin, under the Section 100 (Efficient Funding of Chemotherapy Program), under the same conditions as the PBS-listed bevacizumab biosimilar Mvasi®. The PBAC considered the equi-effective doses of Bevaciptin and Mvasi to be: 100 mg of Bevaciptin = 100 mg of Mvasi and 400 mg of Bevaciptin = 400 mg of Mvasi. The PBAC noted that the listing of Bevaciptin on the PBS is expected to be cost neutral and its listing is not expected to increase the overall use of bevacizumab on the PBS. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| BIMEKIZUMAB<br><br>Solution for injection 160 mg in 1 mL pre-filled pen<br>Solution for injection 160 mg in 1 mL pre-filled syringe<br><br>Bimzelx®<br><br>UCB Australia Proprietary Limited<br><br>Category 2 submission<br>(New PBS listing) | Plaque psoriasis | To request a General Schedule Authority Required (Written) listing for the treatment of adults with severe plaque psoriasis. | Recommended | The PBAC recommended the Authority Required listing of bimekizumab (BKZ) for the treatment of severe chronic plaque psoriasis (CPP). The PBAC considered that, based on the evidence presented in the submission, BKZ was likely to be superior to adalimumab, tildrakizumab and ustekinumab and non-inferior to guselkumab, ixekizumab, secukinumab and risankizumab in terms of achieving Psoriasis Area and Severity Index (PASI) 75 and PASI 90 responses at 12 and 16 weeks respectively. The PBAC noted comparisons versus infliximab, one of the more effective treatments for severe CPP, were not presented in the submission. The PBAC further noted the long-term impact of the differences observed in PASI 75 and 90 at 12 and 16 weeks is unknown. The PBAC recalled that guselkumab, ixekizumab and risankizumab were listed for CPP on the basis of cost-minimisation to the least costly alternative biological disease-modifying antirheumatic drug (bDMARD) and, given the PBAC considered BKZ was likely to be non-inferior to these medicines, it was appropriate to list BKZ on the same basis. The PBAC noted flow-on changes to the currently listed bDMARDs will be required to include BKZ in the list of eligible treatments as part of a treatment cycle. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| BORTEZOMIB<br><br>Solution for injection 2.5 mg<br>Solution for injection 3.5 mg<br><br>Bortezomib Ever Pharma®<br><br>Interpharma Pty Ltd<br><br>Committee secretariat submission<br>(New PBS listing) | Multiple Myeloma | To request a Section 100 (Efficient Funding of Chemotherapy Program) listing of a new form under the same conditions as the currently listed brands of bortezomib. | Recommended | The PBAC recommended the Section 100 (Efficient Funding of Chemotherapy Program) listing of two new forms of bortezomib (Bortezomib Ever Pharma) 2.5 mg/mL and 3.5 mg/1.4 mL solutions for injection under the same circumstances as the currently listed brands of bortezomib. The PBAC noted that the derived price of the new forms is consistent with the other strengths of bortezomib and that the proposed listing is expected to be cost neutral to Government. |
| BUROSUMAB<br><br>Injection 10 mg in 1 mL<br>Injection 20 mg in 1 mL<br>Injection 30 mg in 1 mL<br><br>Crysvita®<br><br>Kyowa Kirin Australia Pty Ltd<br><br>Standard re-entry submission<br>(New PBS listing) | X-linked hypophosphataemia | Resubmission to request a Section 100 (Highly Specialised Drugs Program) Authority Required (Written) listing for the treatment of X-linked hypophosphataemia. | Not Recommended | The PBAC did not recommend burosumab for the treatment of paediatric and adult patients with X-linked hypophosphataemia (XLH). The PBAC noted the high clinical need and strong consumer support for treatments for this condition. However, the PBAC considered that the incremental cost-effectiveness ratio (ICER) was unacceptably high at the proposed price. The PBAC considered a risk sharing arrangement (RSA) was needed to manage the financial impact, including the financial impact associated with use of higher doses.<br><br>The previous submission was considered in March 2021.<br><br>The PBAC nominated the Early Resolution resubmission pathway for this item.<br><br><u>Comparator: conventional therapy consisting or oral phosphorus and calcitriol</u><br><br>The PBAC considered that the nominated comparator was appropriate for both the paediatric and adult populations. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| | | | | Paediatric population:<br><br>Clinical claim: superior effectiveness and safety compared with conventional therapy<br><br>The clinical claim was based on one randomised controlled trial (CL301) comparing burosumab with conventional therapy and three non-comparative studies (CL201, CL205 and KRN23-003).<br><br>The PBAC considered that the claim of superior effectiveness was supported, noting that no data were presented for children aged 13-18 years and the lack of long term follow-up data.<br><br>The PBAC considered that claim of superior safety was not supported. The PBAC considered that burosumab had a different safety profile compared to conventional therapy.<br><br>Economic claim: cost-utility versus conventional therapy<br><br>The economic analysis remained uncertain and the ICER was very high and was likely underestimated.<br><br>Adult population:<br><br>Clinical claim: superior effectiveness and inferior (and acceptable) safety compared with placebo<br><br>The clinical claim was based on one randomised controlled trial (CL303) comparing burosumab with placebo and six non-comparative studies (BUR02, CL304, KRN-INT-001, KRN-INT-002, KRN-US-02 and CL203).<br><br>The PBAC considered that the claim of superior effectiveness was supported, noting the lack of long term follow-up data and comparative evidence |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| | | | | comparing burosumab with the nominated comparator.<br><br>The PBAC considered that claim of inferior (and acceptable) safety was supported.<br><br><u>Economic claim: cost-utility versus conventional therapy</u><br><br>The economic analysis was uncertain and the ICER was very high and was likely underestimated.<br><br><u>Sponsor Comment:</u><br>While disappointed by the decision of the PBAC not to recommend the reimbursement of Crysvita® (burosumab) for the treatment of children and adults with X-linked hypophosphataemia (XLH), Kyowa Kirin considers the offer of an early resolution pathway to be a positive step forward. |
| CABAZITAXEL<br><br>Solution concentrate for I.V. infusion 60 mg in 3 mL<br><br>Cabazitaxel Accord®<br><br>Accord Healthcare Pty. Ltd.<br><br>Committee secretariat submission (New PBS listing) | Prostate cancer | To request a Section 100 (Efficient Funding of Chemotherapy Program) listing of a new form under the same conditions as the currently listed brands of cabazitaxel. | Recommended | The PBAC recommended the Section 100 (Efficient Funding of Chemotherapy) listing of a new form of cabazitaxel (Cabazitaxel Accord) under the same circumstances as the currently listed brands of cabazitaxel for treatment of castration resistant metastatic carcinoma of the prostate. |

| | | | | |
|---|---|---|---|---|
| CANNABIDIOL<br><br>Oral liquid 100 mg per mL, 100 mL<br><br>Epidyolex®<br><br>Chiesi Australia Pty Ltd<br><br>Standard re-entry submission (Change to PBS listing) | Lennox-Gastaut syndrome | To request that the PBAC reconsider a General Schedule Authority Required listing for the adjunctive treatment of seizures in patients with Lennox-Gastaut syndrome (LGS) aged 2 years and older. | Not Recommended | The PBAC did not recommend the listing of cannabidiol for the treatment of LGS in patients who have not achieved adequate seizure control with at least two other anti-epileptic drugs (AEDs), as the incremental cost effectiveness ratio (ICER) was high and uncertain at the requested price.<br><br>The previous submission was considered in July 2020 and November 2020.<br><br><u>Comparator: Standard care (unchanged from previous submission).</u><br><br>The PBAC reaffirmed this was reasonable.<br><br><u>Clinical claim: Cannabidiol plus standard care is superior in terms of comparative effectiveness and inferior comparative safety to placebo plus standard care (unchanged from previous submission).</u><br><br>The PBAC reaffirmed its previous advice that cannabidiol appears to be effective for the treatment of seizures in LGS.<br><br>The PBAC reaffirmed its previous advice that cannabidiol was of inferior comparative safety to placebo.<br><br><u>Economic claim: Cost utility analysis of cannabidiol plus standard care versus placebo plus standard care.</u><br><br>The PBAC considered that the ICER of cannabidiol under its preferred scenario was unacceptably high ($75,000 to < $95,000 per quality adjusted life year) at the requested price. On that basis, the PBAC considered a price reduction to achieve an acceptable ICER would be required to achieve a cost-effective listing for cannabidiol in LGS. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| | | | | The PBAC nominated the early re-entry re-submission pathway for this item.<br><br><u>Sponsor Comment:</u><br>The sponsor is disappointed at the PBAC's decision. The sponsor has very limited room to offer price reductions to achieve an ICER acceptable to the PBAC. Listing Epidyolex on the PBAC will require flexibility on both sides to make Epidyolex available on the PBS for patients with LGS. |
| CARFILZOMIB<br><br>Powder for injection 10 mg<br>Powder for injection 30 mg<br>Powder for injection 60 mg<br><br>Kyprolis®<br><br>Amgen Australia Pty Limited<br><br>Standard re-entry submission (Change to PBS listing) | Multiple myeloma | Resubmission to request a Section 100 (Efficient Funding of Chemotherapy Program) Authority Required (STREAMLINED) listing, for use in combination with lenalidomide and dexamethasone, for the treatment of relapsed refractory multiple myeloma. | Recommended | The PBAC recommended the listing of carfilzomib in combination with lenalidomide and dexamethasone (CLd) for the treatment of relapsed or refractory multiple myeloma. Listing was recommended on the basis of a cost-minimisation approach versus carfilzomib in combination with dexamethasone, and inclusion in the existing carfilzomib risk sharing arrangement (RSA) without an increase in the expenditure caps. The PBAC noted that flow-on restriction changes would be required to create a separate lenalidomide listing to enable its use in CLd. The PBAC noted the resubmission also requested an increase in the expenditure caps established in the Deed of Agreement in place since carfilzomib was PBS listed in January 2018. The PBAC considered the estimates included in the resubmission were not a reliable basis on which to revise the existing RSA expenditure caps. However, the PBAC acknowledged that future revision to the caps may be appropriate given the changing treatment algorithm for multiple myeloma with the availability of multiple new therapies. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| CEMIPLIMAB<br><br>Solution for I.V. infusion 350 mg in 7 mL<br><br>Libtayo®<br><br>Sanofi-Aventis Australia Pty Ltd<br><br>Standard re-entry submission (New PBS listing) | Squamous cell carcinoma | Resubmission to request a Section 100 (Efficient Funding of Chemotherapy Program) Authority Required (STREAMLINED) listing for the treatment of metastatic or locally advanced cutaneous squamous cell carcinoma, in patients who are not candidates for curative surgery or curative radiation. | Recommended | The PBAC recommended the Authority Required (telephone/online PBS Authorities system) listing of cemiplimab for the treatment of patients with metastatic or locally advanced cutaneous squamous cell carcinoma, on the basis that it should be available only under special arrangements under Section 100 (Efficient Funding of Chemotherapy). The PBAC was satisfied that cemiplimab provides, for some patients, improvement in efficacy over best supportive care with or without chemotherapy. The PBAC considered that, while the magnitude of difference in effectiveness of cemiplimab remains uncertain, there is a high unmet clinical need in this population with potential for quality of life benefits not encompassed in the available data. The PBAC's recommendation for listing was based on, among other matters, its assessment that the subsequent uncertainty in the incremental cost-effectiveness ratio (ICER) could be addressed through a lower ICER threshold and resulting price reduction. The PBAC considered the exclusion of patients with an Eastern Cooperative Oncology Group (ECOG) performance status of 2 or more appropriate and considered the financial estimates should be amended accordingly. In addition, the PBAC considered a risk sharing arrangement appropriate to manage the risk of use beyond the restriction. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| CICLOSPORIN<br><br>Eye drops 900 micrograms per mL, single dose units, 0.25 mL, 60<br><br>Cequa®<br><br>Sun Pharma ANZ Pty Ltd<br><br>Category 2 submission<br>(New PBS listing) | Chronic severe dry eye disease with keratitis | To request a General Schedule Authority Required listing for the treatment of chronic severe dry eye disease with keratitis under the same restrictions as the currently listed ciclosporin eye drops. | Recommended | The PBAC recommended the listing of ciclosporin 0.09% eye drops for the treatment of chronic severe dry eye disease with keratitis. The PBAC's recommendation for listing was based on, among other matters, its assessment that the cost-effectiveness of ciclosporin 0.09% eye drops would be acceptable if it were cost-minimised against ciclosporin 0.1% eye drops. The PBAC considered it would be useful to have an additional formulation of ciclosporin eye drops with a different vehicle to the currently listed ciclosporin 0.1% eye drops which would provide an alternative for patients who may not tolerate this formulation. The PBAC considered it was appropriate for ciclosporin 0.09% eye drops to join the existing risk sharing arrangement currently in place for ciclosporin 0.1% eye drops. The PBAC recommended potential flow-on changes to 0.1% ciclosporin eye drops PBS restriction to prevent concomitant use with ciclosporin 0.09% eye drops. |
| DAPAGLIFLOZIN<br><br>Tablet 10 mg<br><br>Forxiga®<br><br>AstraZeneca Pty Ltd<br><br>Early re-entry submission<br>(Change to PBS listing) | Chronic kidney disease | Resubmission to request a General Schedule Authority Required (STREAMLINED) listing for the treatment of chronic kidney disease. | Recommended | The PBAC recommended the listing of dapagliflozin for the treatment of chronic kidney disease. The PBAC was satisfied that dapagliflozin added to standard care provides, for some patients, a significant improvement in efficacy over standard care alone. The PBAC considered that the listing would be cost-effective at the price proposed in the pre-PBAC response from July 2021. The PBAC considered the financial estimates were uncertain and advised that a risk sharing arrangement would be required. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| DIPHTHERIA, TETANUS, PERTUSSIS, HEPATITIS B, POLIOMYELITIS AND HAEMOPHILUS INFLUENZAE TYPE B CONJUGATE VACCINE (DTPa-HB-IPV-Hib)<br><br>0.5 mL pre-filled syringe<br><br>Vaxelis®<br><br>Sanofi-Aventis Australia Pty Ltd<br><br>Category 2 submission<br>(New listing) | Diphtheria, tetanus, pertussis, hepatitis B, poliomyelitis and Haemophilus influenzae type b | To request National Immunisation Program listing for the prevention of diphtheria, tetanus, pertussis, hepatitis B, poliomyelitis and Haemophilus influenzae type b. | Recommended | The PBAC recommended that diphtheria, tetanus, pertussis, hepatitis B, poliomyelitis and Haemophilus influenzae type b conjugate vaccine (DTPa-HB-IPV-Hib, Vaxelis) be a designated vaccine for the purposes of the *National Health Act 1953*, for the primary vaccination series against diphtheria, tetanus, pertussis, hepatitis B, poliomyelitis and invasive infections caused by Haemophilus influenzae type b at 2, 4 and 6 months of age. The PBAC also considered Vaxelis to be suitable for catch-up for children under 10 years of age. The PBAC's recommendation for listing was based on, among other matters, its assessment that the cost-effectiveness of Vaxelis would be acceptable if it were cost-minimised against the nominated comparator, Infanrix Hexa® (the DTPa-HB-IPV-Hib vaccine currently listed on the National Immunisation Program). |
| DIROXIMEL FUMARATE<br><br>Capsule 231 mg<br><br>Vumerity®<br><br>Biogen Australia Pty Ltd<br><br>Category 2 submission<br>(New PBS listing) | Multiple sclerosis | To request a General Schedule Authority Required (STREAMLINED) listing for the treatment of relapsing-remitting multiple sclerosis. | Recommended | The PBAC recommended the Authority Required (STREAMLINED) listing of diroximel fumarate for the treatment of relapsing-remitting multiple sclerosis. The PBAC considered the claim of non-inferior effectiveness and safety to dimethyl fumarate was reasonable. However, the PBAC considered for purposes of satisfying Section 101(3B) of the *National Health Act 1953*, glatiramer acetate, interferon beta-1a (both subcutaneous and intramuscular forms), interferon beta-1b and peg-interferon beta-1a are relevant alternative therapies. The PBAC's recommendation was therefore, among other matters, based on its assessment that the cost of diroximel fumarate should be no greater than the cost of dimethyl fumarate or the alternative therapies at comparable daily doses. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| DORZOLAMIDE WITH TIMOLOL<br><br>Eye drops containing dorzolamide 20 mg (as hydrochloride) with timolol 5 mg (as maleate) per mL, 5 mL<br><br>VIzo-PF Dorzolatim®<br><br>Aft Pharmaceuticals (AU) Pty Ltd<br><br>Committee secretariat submission (New PBS listing) | Elevated intra-ocular pressure | To request a General Schedule Restricted Benefit listing of a new form in a preservative-free multi-dose bottle under the same conditions as the currently listed brands of dorzolamide with timolol. | Recommended | The PBAC recommended the listing of a new ophthalmic eye drop solution in a preservative-free multi-dose bottle, Vizo-PF Dorzolatim®, under the same circumstances as the currently listed brands of dorzolamide + timolol eye drops (Cosopt® and Cosdor®), for the treatment of elevated intraocular pressure in patients with ocular hypertension or open-angle glaucoma when concomitant therapy is appropriate. The PBAC advised, under Section 101 (4AACD) of the *National Health Act 1953*, that the Vizo-PF Dorzolatim®, Cosopt® and Cosdor® brands of dorzolamide + timolol eye drops should be considered equivalent for the purposes of substitution. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| DOSTARLIMAB<br><br>Solution concentrate for I.V. infusion 500 mg in 10 mL<br><br>Jemperli®<br><br>GlaxoSmithKline Australia Pty Ltd<br><br>Category 1 submission<br>(New PBS listing) | Endometrial cancer | To request a Section 100 (Efficient Funding of Chemotherapy Program) Authority Required (STREAMLINED) listing for the treatment of recurrent or advanced mismatch repair deficient endometrial cancer that has progressed on or following prior treatment with a platinum-containing regimen. | Not Recommended | The PBAC did not recommend the listing of dostarlimab for the treatment of patients with recurrent or advanced mismatch repair deficient endometrial cancer who have disease progression following prior systemic therapy. The PBAC noted that the evidence submitted for dostarlimab was based on a relatively small single arm study with immature follow-up and considered there were key transitivity and methodological issues with the indirect comparisons presented in the submission. Overall, the PBAC considered that the magnitude of benefit for dostarlimab over chemotherapy was uncertain and the incremental cost-effectiveness ratio was highly uncertain because the modelled survival benefit was not adequately supported by the available data.<br><br><u>Sponsor Comment:</u><br>GSK is disappointed by the PBAC's decision not to recommend dostarlimab (Jemperli®) for the treatment of patients with recurrent or advanced mismatch repair deficient endometrial cancer that has progressed on or following prior treatment with a platinum-containing regimen. However, we remain committed to working with the PBAC to ensure Australian women with endometrial cancer have timely access to Jemperli®. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| DULAGLUTIDE<br><br>Injection 3 mg in 0.5 mL single dose pre-filled pen<br>Injection 4.5 mg in 0.5 mL single dose pre-filled pen<br><br>Trulicity®<br><br>Eli Lilly Australia Pty Ltd<br><br>Category 2 submission<br>(New PBS listing) | Type 2 diabetes mellitus | To request General Schedule Authority Required (STREAMLINED) listings of two new forms for the treatment of patients with Type 2 diabetes mellitus who require treatment intensification to achieve glycaemic targets, as dual therapy in combination with metformin. | Deferred | The PBAC deferred making its decision on whether to recommend the listing of dulaglutide 3 mg and 4.5 mg for the treatment of Type 2 diabetes mellitus in combination with metformin, as a TGA Delegate's Overview was not available at the time of PBAC consideration. The PBAC was of a mind to recommend dulaglutide 3 mg and 4.5 mg on a cost-minimisation basis compared to dulaglutide 1.5 mg, pending receipt of a positive TGA Delegate's Overview.<br><br><u>Sponsor Comment:</u><br>The sponsor had no comment. |
| DUPILUMAB<br><br>Injection 200 mg in 1.14 mL single dose pre-filled syringe<br>Injection 300 mg in 2 mL single dose pre-filled syringe<br><br>Dupixent®<br><br>Sanofi-Aventis Australia Pty Ltd<br><br>Category 2 submission<br>(Change to PBS listing) | Atopic dermatitis | To request a General Schedule Authority Required listing for the treatment of severe atopic dermatitis in patients aged 6 to 11 years. | Recommended | The PBAC recommended extending the listing of dupilumab to include patients aged less than 12 years with severe atopic dermatitis. The PBAC noted the substantial clinical need for effective treatments for these patients and was satisfied that dupilumab provides, for some patients, a significant improvement in efficacy over standard care. The PBAC considered that the clinical evidence suggests the magnitude of benefit in children is similar to that in the adult/adolescent population and the cost-effectiveness was acceptable at the same price per month as for the adult/adolescent population. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| DURVALUMAB<br><br>Solution concentrate for I.V. infusion 500 mg in 10 mL<br><br>Imfinzi®<br><br>AstraZeneca Pty Ltd<br><br>Category 3 submission<br>(Change to PBS listing) | Unresectable Stage III, non-small cell lung cancer | To request a Section 100 (Efficient Funding of Chemotherapy Program) Authority Required (STREAMLINED) listing of a new maximum amount of 1500 mg with four repeats to allow an additional dosing regimen of durvalumab to allow a fixed-dose 1500 mg every 4 weeks. | Recommended | The PBAC recommended an amendment to the listing of the durvalumab on the PBS to provide clinicians with the option of a flat dosing regimen of 1500 mg every four weeks, in addition to the existing weight-based dosing regimen, for the treatment of non-small cell lung cancer. The PBAC recommended the following amendments be made to the existing listing to achieve this:<br>• increase the maximum amount from 1200 mg to 1500 mg.<br>• reduce the number of repeats from 8 to 4.<br>• remove the administrative advice: 'No increase in the maximum number of repeats may be authorised'. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| ENFORTUMAB VEDOTIN<br><br>Powder for I.V. infusion 20 mg<br>Powder for I.V. infusion 30 mg<br><br>Padcev®<br><br>Astellas Pharma Australia Pty Ltd<br><br>Category 1 submission<br>(New PBS listing) | Urothelial cancer | To request a Section 100 (Efficient Funding of Chemotherapy Program) Authority Required (STREAMLINED) listing for the treatment of locally advanced (Stage III) or metastatic (Stage IV) urothelial cancer in patients with a World Health Organisation (WHO) performance status of 0 or 1 and who have progressed on or after treatment with a platinum-containing chemotherapy regimen and either a programmed cell death-1 (PD-1) inhibitor or a programmed cell death ligand-1 (PD-L1) inhibitor. | Not Recommended | The PBAC did not recommend enfortumab vedotin for the treatment of patients with locally advanced (Stage III) or metastatic (Stage IV) urothelial cancer who have progressed on or after a platinum-containing chemotherapy regimen and either a programmed cell death-1 (PD-1) inhibitor or a programmed cell death ligand-1 (PD-L1) inhibitor. The PBAC considered data from the key trial EV-301 supported a moderate overall survival benefit. In addition, the PBAC considered the evidence presented suggested that enfortumab vedotin had an acceptable safety profile. However, the PBAC considered the incremental cost-effectiveness ratio (ICER) proposed in the submission was uncertain and the respecified ICERs were high at the proposed price.<br><br>The PBAC nominated the Early Re-entry re-submission pathway for this item.<br><br><u>Sponsor Comment:</u><br>Astellas Pharma Australia will continue to work with the PBAC to seek to resolve the outstanding issues so patients in Australia with locally advanced (Stage III) or metastatic (Stage IV) urothelial cancer can benefit from enfortumab vedotin being available through the PBS. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| ENZALUTAMIDE<br><br>Capsule 40 mg<br><br>Xtandi®<br><br>Astellas Pharma Australia Pty Ltd<br><br>Category 2 submission<br>(Change to PBS listing) | Prostate cancer | To request a General Schedule Authority Required listing, in combination with ongoing androgen-deprivation therapy, for the treatment of non-metastatic castration-resistant prostate cancer (m0CRPC). | Recommended | The PBAC recommended the listing of enzalutamide for the treatment of patients with m0CRPC. The PBAC was satisfied that enzalutamide was non-inferior in terms of efficacy and safety compared to darolutamide, the primary comparator. The PBAC's recommendation for listing was based on, among other matters, its assessment that the cost-minimisation approach between enzalutamide and darolutamide was acceptable and that the listing would be cost-neutral to Government. The PBAC recommended potential flow-on changes to the PBS restrictions for abiraterone and enzalutamide in the metastatic CRPC setting to prevent their use subsequent to enzalutamide in the m0CRPC setting. |
| FREMANEZUMAB<br><br>Solution for injection 225 mg in 1.5 mL single dose auto-injector<br><br>Ajovy®<br><br>Teva Pharma Australia Pty Ltd<br><br>Committee secretariat submission<br>(New PBS listing) | Chronic migraine | To request General Schedule Authority Required (STREAMLINED) listing of a new form under the same conditions as the PBS-listed fremanezumab pre-filled syringes. | Recommended | The PBAC recommended the Authority Required (STREAMLINED) listing of fremanezumab (Ajovy) 225 mg in 1.5 mL auto-injector under the same circumstances as the PBS-listed fremanezumab (Ajovy) 225 mg in 1.5 mL pre-filled syringe. The PBAC advised that, under section 101(4AACD) of the *National Health Act 1953*, in the Schedule of Pharmaceutical Benefits, fremanezumab auto-injector and fremanezumab pre-filled syringe should be treated as equivalent ('a'-flagged) to each other for the purposes of substitution. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| FREMANEZUMAB<br><br>Solution for injection 225 mg in 1.5 mL single dose pre-filled syringe<br>Solution for injection 225 mg in 1.5 mL single dose auto-injector<br><br>Ajovy®<br><br>Teva Pharma Australia Pty Ltd<br><br>Category 4 submission<br>(Change to PBS listing) | Chronic migraine | To request General Schedule Authority Required (STREAMLINED) listing with maximum quantity of three and one repeat to allow quarterly dosing in addition to the existing monthly dosing. | Recommended | The PBAC recommended amending the existing listing of fremanezumab (Ajovy) pre-filled syringe for the continuing treatment of chronic migraine to provide patients with options of both monthly dosing and quarterly dosing by increasing the maximum quantity from 1 to 3 and reducing the number of repeats from 5 to 1. The PBAC recommended these restriction changes to be flowed on to the auto-injector form. |
| GALCANEZUMAB<br><br>Injection 120 mg in 1 mL pre-filled pen<br><br>Emgality®<br><br>Eli Lilly Australia Pty Ltd<br><br>Standard re-entry submission<br>(Change to PBS listing) | Migraine | Resubmission to extend the current General Schedule Authority Required (STREAMLINED) listing to include treatment-resistant high frequency episodic migraine. | Recommended | The PBAC recommended amending the current PBS listing of galcanezumab for chronic migraine to include the treatment of patients with high frequency episodic migraine by removing the criteria for patients to have an average of 15 or more headache days per month. The resulting PBS listing for galcanezumab is for the treatment of patients who have an inadequate response, intolerance, or a contraindication to at least three prophylactic migraine medications, with 8 or more migraine headache days per month. The PBAC considered galcanezumab would be cost effective for the high frequency episodic migraine patient population at a price no higher than the price for patients with chronic migraine. The PBAC considered the new patient population should be included in the chronic migraine risk sharing arrangement with an increase in expenditure caps. Minor flow-on changes to the fremanezumab restriction criteria will be required to address an inconsistency between the current initial and continuing restriction criteria for chronic migraine. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| GILTERITINIB<br><br>Tablet 40 mg (as fumarate)<br><br>Xospata®<br><br>Astellas Pharma Australia Pty Ltd<br><br>Early re-entry submission<br>(New PBS listing) | Acute myeloid leukaemia | Resubmission to request a General Schedule Authority Required listing for the treatment of relapsed or refractory FMS-like tyrosine kinase 3 (FLT3) mutation-positive acute myeloid leukaemia. | Recommended | The PBAC recommended the Authority Required listing of gilteritinib for the treatment of patients with relapsed or refractory acute myeloid leukaemia (AML) with an FLT3 mutation. The PBAC was satisfied that gilteritinib provides, for some patients, a significant improvement in efficacy over salvage chemotherapy. The PBAC considered that the resubmission had addressed the outstanding issues identified at the November 2021 PBAC meeting via its respecified economic model and revised financial estimates, which aligned with the PBAC's previous advice. At the reduced price offered, the listing was expected to be cost-effective in comparison with salvage chemotherapy. |
| GLATIRAMER<br><br>Injection containing glatiramer acetate 40 mg in 1 mL single dose pre-filled pen<br><br>Copaxone®<br><br>Teva Pharma Australia Pty Ltd<br><br>Committee secretariat<br>(New PBS listing) | Multiple sclerosis | To request a General Schedule Authority Required (STREAMLINED) listing of a new form under the same conditions as the PBS-listed glatiramer pre-filled syringe. | Recommended | The PBAC recommended the listing of a new form of 40 mg glatiramer acetate pre-filled pen under the same circumstances as the PBS-listed 40 mg glatiramer pre-filled syringe for the treatment of multiple sclerosis. The PBAC also decided to advise that the 40 mg glatiramer acetate pre-filled pen form should be treated as equivalent to the 40 mg glatiramer acetate pre-filled syringe form for the purpose of substitution (i.e. 'a'-flagged in the Schedule) |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| GLYCOMACROPEPTIDE FORMULA WITH AMINO ACIDS, CARBOHYDRATES, MINERALS AND LOW PHENYLALANINE<br><br>Sachets containing oral powder 12.5 g, 30 (PKU GMPro Mix-In)<br><br>PKU GMPro MIX-IN®<br><br>Nutricia Australia Pty Limited<br><br>Category 3 submission<br>(New PBS listing) | Phenylketonuria | To request a General Schedule Restricted Benefit listing for treatment of phenylketonuria in patients older than 3 years of age. | Recommended | The PBAC recommended the General Schedule Restricted Benefit listing of glycomacropeptide formula with amino acids and low phenylalanine (PKU GMPro MIX-IN®) for the treatment of phenylketonuria. |
| GLYCOMACROPEPTIDE FORMULA WITH AMINO ACIDS, VITAMINS, MINERALS, TRACE ELEMENTS, CARBOHYDRATE, FAT AND LOW PHENYLALANINE<br><br>Sachets containing oral powder 33.4 g, 30 (PKU GMPro ULTRA)<br><br>PKU GMPro ULTRA®<br><br>Nutricia Australia Pty Limited<br><br>Category 3 submission<br>(New PBS listing) | Phenylketonuria | To request a General Schedule Restricted Benefit listing for treatment of phenylketonuria in patients older than 3 years of age. | Recommended | The PBAC recommended the General Schedule Restricted Benefit listing of glycomacropeptide formula with amino acids, vitamins, minerals, trace elements, carbohydrate, fat and low phenylalanine (PKU GMPro ULTRA®) for the treatment of phenylketonuria. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| INFLIXIMAB<br><br>Solution for injection 120 mg in 1 mL pre-filled pen<br>Solution for injection 120 mg in 1 mL pre-filled syringe<br><br>Remsima® SC<br><br>Celltrion Healthcare Australia Pty Ltd<br><br>Category 3 submission<br>(New PBS listing) | Ankylosing spondylitis<br>Psoriatic arthritis<br>Chronic plaque psoriasis<br>Refractory fistulising Crohn Disease | To request a General Schedule Authority Required listing for the treatment of ankylosing spondylitis, psoriatic arthritis, chronic plaque psoriasis and refractory fistulising Crohn Disease. | Recommended | The PBAC recommended the listing of infliximab subcutaneous injections for the treatment of ankylosing spondylitis, severe chronic plaque psoriasis, severe active psoriatic arthritis and complex refractory fistulising Crohn's Disease on a cost-minimisation basis to infliximab intravenous injection forms. |
| IXAZOMIB<br><br>Capsule 2.3 mg<br>Capsule 3 mg<br>Capsule 4 mg<br><br>Ninlaro®<br><br>Takeda Pharmaceuticals Australia Pty Ltd<br><br>Standard re-entry<br>(New PBS listing) | Multiple myeloma | Resubmission to request a Section 100 (Highly Specialised Drugs Program) Authority Required listing, in combination with lenalidomide and dexamethasone, for the treatment of relapsed and/or refractory multiple myeloma in patients who have received at least two prior therapies. | Not Recommended | The PBAC did not recommend the listing of ixazomib, for use in combination with lenalidomide and dexamethasone (ILd), for the treatment of relapsed and/or refractory multiple myeloma (RRMM) in patients who have received at least two prior therapies.<br><br>The previous submission was considered in November 2020.<br><br><u>Comparator: lenalidomide plus dexamethasone (Ld)</u><br><br>The PBAC considered that Ld was a relevant comparator; however, noted that the multiple treatment options for RRMM and the changing treatment algorithm complicated the selection of the main comparator(s). The PBAC considered that Carfilzomib in combination with dexamethasone (Cd) remained an important comparator.<br><br><u>Clinical claim: superior effectiveness and non-inferior safety compared with Ld</u><br><br>The clinical claim was based on the TOURMALINE MM-1 (TMM-1). Updated overall survival (OS) data |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| | | | | was presented compared to the November 2020 submission.<br><br>The PBAC considered that the claim of superior effectiveness was not supported. Although ILd resulted in a statistically significant improvement in progression free survival (PFS) compared to Ld for the intention to treat (ITT) population and for patients who had received 2-3 lines of prior therapy, the overall survival (OS) results were not statistically significant for the ITT population or for the 2-3 prior lines of therapy subgroup.<br><br>The PBAC considered that the non-inferior safety claim was reasonable.<br><br><u>Economic claim: cost-utility versus Ld</u><br><br>The economic analysis was uncertain, as it relied on a gain in OS, and was unacceptably high.<br><br><u>Sponsor Comment:</u><br>The sponsor had no comment. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| LAROTRECTINIB<br><br>Capsule 25 mg<br>Capsule 100 mg<br>Oral solution 20 mg per mL, 100 mL<br><br>Vitrakvi®<br><br>Bayer Australia Ltd<br><br>Matters outstanding<br>(New PBS listing) | Solid tumours harbouring neurotrophic receptor tyrosine kinase (NTRK) gene fusions | Resubmission to request a Section 100 (Highly Specialised Drugs Program) Authority Required (Written) listing for the treatment of adult and paediatric patients with locally advanced or metastatic solid tumours harbouring NTRK gene fusions. | Recommended | The PBAC recommended the listing of larotrectinib for the treatment of patients with tropomyosin receptor kinase (NTRK) fusion tumours that are either unresectable locally advanced, metastatic, or locally advanced and unsuitable for surgery (i.e. would otherwise require disfiguring surgery or limb amputation to achieve a complete surgical resection) under the Section 85 (General Schedule). The PBAC recommended the listing for paediatric patients with NTRK fusion tumours and adult patients with high frequency NTRK fusion tumours (specifically mammary analogue secretory carcinoma and secretory breast carcinoma) on the basis that the incremental cost-effectiveness ratio was acceptable at the proposed price and noting that MSAC supported funding the co-dependent NTRK testing to determine eligibility for treatment with larotrectinib in all paediatric patients, and adult patients with high-frequency NTRK fusion cancer types. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| MECASERMIN<br><br>Solution for injection 40 mg in 4 mL vial<br><br>Increlex®<br><br>Ipsen Pty Ltd<br><br>Early re-entry submission<br>(New PBS listing) | Primary insulin-like growth factor 1 deficiency | Resubmission to request a Section 100 (Growth Hormone Program) Authority Required (Written) listing for the treatment of children and adolescents with growth failure due to primary insulin-like growth factor 1 deficiency. | Recommended | The PBAC recommended the listing of mecasermin on the basis that it be available on a Section 100 (Growth Hormone Program) listing for the long-term treatment of growth failure in children and adolescents from 2 to 18 years with severe primary insulin-like growth factor 1 deficiency. The PBAC was satisfied that mecasermin provides, for some patients, a significant improvement in efficacy, based on improved height outcomes over the nominated comparator, no treatment. The PBAC considered that the expert advice provided by the Australasian Paediatric Endocrine Group (APEG) had resolved the majority of concerns raised at the November 2021 PBAC meeting, and the remaining concerns could be addressed with an appropriate risk sharing arrangement. The PBAC noted that additional communication with APEG would be required to finalise the restrictions. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| METHYLPHENIDATE<br><br>Capsule containing methylphenidate hydrochloride 10 mg (modified release)<br>Capsule containing methylphenidate hydrochloride 20 mg (modified release)<br>Capsule containing methylphenidate hydrochloride 30 mg (modified release)<br>Capsule containing methylphenidate hydrochloride 40 mg (modified release)<br>Capsule containing methylphenidate hydrochloride 60 mg (modified release)<br><br>Ritalin® LA<br><br>Novartis Pharmaceuticals Australia Pty Limited<br><br>Category 3 submission<br>(Change to PBS listing) | Attention deficit hyperactivity disorder | To request a General Schedule Authority Required listing for the treatment of adult patients with attention deficit hyperactivity disorder under the same population criteria as the currently listed lisdexamfetamine (LDX) for adult population. | Recommended | The PBAC recommended expanding the listing of methylphenidate (Ritalin® LA) to include treatment of patients with attention deficit hyperactivity disorder who are diagnosed after the age of 18. The PBAC considered that listing of Ritalin® LA in the broader population would be cost-effective at a reduced price that is lower than that of LDX, and in order to achieve, at least in part, the submission's estimated cost savings in the context of anticipated market growth. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| NIRAPARIB<br><br>Capsule 100 mg<br><br>Zejula®<br><br>GlaxoSmithKline Australia Pty Ltd<br><br>Standard re-entry submission<br>(New PBS listing) | High grade epithelial ovarian, fallopian tube, or primary peritoneal cancer | Resubmission to request a General Schedule Authority Required (STREAMLINED) listing for the treatment of newly diagnosed, advanced, high grade epithelial ovarian, fallopian tube, or primary peritoneal cancer that is responsive (complete/partial) to platinum-based chemotherapy. | Recommended | The PBAC recommended niraparib for the treatment of high-grade epithelial ovarian, fallopian tube, or primary peritoneal cancer in patients with BRCA1/2 pathogenic gene variants, who are in response (complete response or partial response) to first line (1L) platinum-based chemotherapy. The PBAC's recommendation for listing was based on, among other matters, its assessment that the cost-effectiveness of niraparib would be acceptable if it were cost-minimised to olaparib. The PBAC did not recommend niraparib for patients without evidence of a BRCA1/2 pathogenic gene variant. The PBAC considered that in this population the progression-free survival benefit from treatment with niraparib was relatively small (3.5 months) and may not result in an overall survival benefit. The PBAC considered that for a number of patients in this population there may be no clinical benefit from treatment with niraparib, and added toxicity. The PBAC considered that the cost-effectiveness of niraparib was uncertain because the economic model relied on gains in overall survival that were not supported by the immature trial data. |
| NIVOLUMAB<br><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>Bristol-Myers Squibb Australia Pty Ltd<br><br>Category 3 submission<br>(Change to PBS listing) | Second-line squamous cell oesophageal carcinoma | To request the PBAC to reconsider its July 2021 recommendation for a Section 100 (Efficient Funding of Chemotherapy Program), Authority Required (STREAMLINED) listing for second-line treatment of patients with squamous cell oesophageal carcinoma who have failed treatment with a fluoropyrimidine and platinum containing treatment regimen. | Recommended | The PBAC recommended nivolumab for the second-line treatment of patients with advanced or metastatic oesophageal squamous cell carcinoma who have failed one fluoropyrimidine and platinum-based chemotherapy treatment regimen and considered it would be cost-effective at the same price per 100 mg vial recommended for the first line treatment of gastro-oesophageal cancers. The PBAC considered it would be appropriate to implement a single listing for gastro-oesophageal cancers including the first and second- line populations. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| NIVOLUMAB<br><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>Bristol-Myers Squibb Australia Pty Ltd<br><br>Early resolution submission<br>(Change to PBS listing) | Non-HER-2-positive gastric cancer, gastroesophageal junction cancer or oesophageal adenocarcinoma | Resubmission to request a Section 100 (Efficient Funding of Chemotherapy Program) Authority Required (STREAMLINED) listing for the first-line treatment of patients with advanced or metastatic non-HER-2-positive gastric cancer, gastroesophageal junction cancer or oesophageal adenocarcinoma. | Recommended | The PBAC recommended the listing of nivolumab in combination with chemotherapy for the first line treatment of advanced or metastatic gastro-oesophageal cancers as defined by the specific tumour types included in the approved TGA registered indications. The PBAC considered the early resolution resubmission had appropriately addressed the outstanding issues as outlined in the November 2021 PBAC minutes. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| NUSINERSEN<br><br>Solution for injection 12 mg in 5 mL<br><br>Spinraza®<br><br>Biogen Australia Pty Ltd<br><br>Standard re-entry submission (Change to PBS listing) | Spinal muscular atrophy | Resubmission to request a Section 100 (Highly Specialised Drugs Program) Authority Required (Written) listing for the treatment of adults diagnosed with 5q spinal muscular atrophy (SMA) with symptom onset prior to 19 years of age (≤18 years of age). | Recommended | The PBAC recommended the listing of nusinersen for the treatment of adult patients (older than 18 years of age) diagnosed with SMA with symptom onset before 19 years of age (primarily SMA Types II and III), on the basis that it should be available only under special arrangements under Section 100 (Highly Specialised Drugs Program). The PBAC is satisfied that nusinersen provides, for some patients, a significant improvement in efficacy over standard care. The PBAC recognised the clinical need for effective treatments for adults with SMA. The PBAC noted the comprehensive input received from adults living with SMA, including individual submissions, and through the SMA Australia member survey. This information was detailed and important for the PBAC's deliberations. The PBAC also noted the important input received from clinical specialists, including the comprehensive work done on development of Australian guidelines for managing treatment of SMA in adult patients. The PBAC noted that the changes to the restrictions proposed in the resubmission were based on consultation with patients and clinicians and considered this helped to ensure the adult population most likely to benefit from treatment was sufficiently defined. The PBAC also noted that the sponsor proposed a substantial price reduction in the pre-PBAC response to better reflect the benefit of treatment in adult SMA patients. The sponsor also proposed a risk sharing arrangement which accounts for the number of patients treated and the number of patients who respond to treatment. The PBAC considered that a review of uptake and continuation rates in adult SMA patients should take place within three years after listing. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| OZANIMOD<br><br>Capsule 920 micrograms<br>Pack containing 4 capsules<br>230 micrograms and 3 capsules<br>460 micrograms<br><br>Zeposia®<br><br>Celgene Pty Limited<br><br>Category 2 submission<br>(Change to PBS listing) | Ulcerative colitis | To request a General Schedule Authority Required (Written) listing for the treatment of moderate to severe ulcerative colitis (MSUC). | Recommended | The PBAC recommended the General Schedule, Authority Required (Written) listing of ozanimod (OZA) for the treatment of MSUC on a cost-minimisation basis with the least costly alternative disease-modifying anti-rheumatic drug (DMARD). In making this recommendation, the PBAC considered that, whilst there were numerous uncertainties with the presented evidence and clinical claim due to differences in trial designs and recruited populations between therapies, that a claim of non-inferior comparative effectiveness and safety to infliximab was, on balance, likely to be reasonable. The PBAC noted that there was no basis for a price premium over alternative therapies. The PBAC noted the flow-on changes to other DMARD listings in MSUC to include OZA in the list of eligible treatments in a treatment cycle. |
| PALBOCICLIB<br><br>Tablet 75 mg<br>Tablet 100 mg<br>Tablet 125 mg<br><br>Ibrance®<br><br>Pfizer Australia Pty Ltd<br><br>Category 2 submission<br>(Change to recommended PBS listing) | Breast cancer | To request a General Schedule Authority Required listing, for use in combination with fulvestrant, for the treatment of hormone receptor positive (HR+) and human epidermal growth factor receptor 2 negative (HER2-) locally advanced inoperable or metastatic breast cancer in patients who have received prior endocrine therapy. | Recommended | The PBAC recommended the listing of palbociclib, in combination with fulvestrant, for the treatment of patients with locally advanced (stage IIIB/IIIC) or metastatic (stage IV) HR+, HER2- breast cancer who have received previous endocrine therapy. The PBAC's recommendation for listing was based on, among other matters, its assessment that the cost-minimisation approach between palbociclib plus fulvestrant and ribociclib and fulvestrant was acceptable and that the listing was expected to be cost-netural to Government. The PBAC advised that flow-on restriction changes would be required for ribociclib and abemaciclib to prevent the sequential use of cyclin-dependent kinase 4 and 6 (CDK4/6) inhibitors. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| PALIPERIDONE<br><br>I.M. injection (modified release) 700 mg (as palmitate) in pre-filled syringe<br>I.M. injection (modified release) 1000 mg (as palmitate) in pre-filled syringe<br><br>Invega Hafyera®<br><br>Janssen-Cilag Pty Ltd<br><br>Category 2 submission<br>(New PBS listing) | Schizophrenia | To request a General Schedule Authority Required (STREAMLINED) listing for the maintenance treatment of schizophrenia in patients who have been stabilised on either PBS-subsidised paliperidone three-monthly injection for at least one injection cycle or PBS-subsidised paliperidone once-monthly for at least four consecutive months. | Recommended | The PBAC recommended the listing of paliperidone palmitate 6-monthly long-acting injection (PP6M) for the maintenance treatment of schizophrenia in adult patients who have been adequately treated with the 1-month paliperidone palmitate injectable product (PP1M) for at least four months or the 3-month paliperidone palmitate injectable product (PP3M) for at least one 3-month injection cycle. The PBAC's recommendation for listing was based on, among other matters, its assessment that the cost-effectiveness of PP6M would be acceptable if it were cost-minimised to PP3M for the same indication. The PBAC considered the equi-effective doses to be 1 injection of PP6M 700 mg = 2 injections of PP3M 350 mg and 1 injection of PP6M 1000 mg = 2 injections of PP3M 525 mg over 48 weeks. The PBAC considered that the restriction for PP6M should be consistent with the restriction for PP3M with the inclusion of the criterion of being stabilised on PP3M for at least one 3-month injection cycle. The PBAC advised that there should be flow-on changes to allow patients to transition from PP6M to PP3M or PP1M if they have been stabilised on PP6M for at least one 6-month injection cycle. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| PEGCETACOPLAN<br><br>Solution for subcutaneous infusion 1,080 mg in 20 mL<br><br>Empaveli™<br><br>Swedish Orphan Biovitrum Pty Ltd<br><br>Category 2 submission<br>(New PBS listing) | Paroxysmal nocturnal haemoglobinuria | To request a Section 100 (Highly Specialised Drugs Program) Authority Required (Written) listing for the treatment of adults with paroxysmal nocturnal haemoglobinuria who have an inadequate clinical response to complement component 5 (C5) inhibitor treatment. | Not Recommended | The PBAC did not recommended pegcetacoplan, for the treatment of adults with paroxysmal nocturnal haemoglobinuria who have inadequate clinical response to treatment with eculizumab or ravulizumab. The PBAC considered the evidence presented demonstrated a benefit compared to eculizumab in short-term haematological outcomes. However, the PBAC considered the economic model provided was not reliable for decision-making and hence cost-effectiveness could not be assessed. The PBAC considered a cost-minimisation approach would likely be appropriate in any resubmission with revised financial estimates.<br><br>The PBAC nominated the Early Re-entry resubmission pathway for this item.<br><br><u>Sponsor Comment:</u><br>The sponsor had no comment. |
| 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>Matters outstanding<br>(Change to PBS listing) | Squamous cell carcinoma of the head and neck (SCCHN) | Resubmission to request a Section 100 (Efficient Funding of Chemotherapy Program) Authority Required (STREAMLINED) listing for the first-line treatment of recurrent or metastatic squamous cell carcinoma of the head and neck (R/M SCCHN). | Recommended | The PBAC recommended listing of pembrolizumab monotherapy for first line treatment of R/M SCCHN patients with combined positive score (CPS) ≥20 in their tumour sample, and pembrolizumab in combination with platinum-based chemotherapy irrespective of CPS score. The PBAC is satisfied that pembrolizumab provides, for some patients, a significant improvement in efficacy over chemotherapy alone. The PBAC noted that the MSAC supported its preferred approach of a CPS ≥20 threshold for pembrolizumab monotherapy and an all-comers population for pembrolizumab plus chemotherapy. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| 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>Category 1 submission<br>(Change to PBS listing) | Endometrial cancer | To request a Section 100 (Efficient Funding of Chemotherapy Program) Authority Required listing, in combination with lenvatinib, for the treatment of patients with advanced endometrial cancer (regardless of biomarker status) who have disease progression following prior systemic therapy.<br><br>The submission also requests a listing for pembrolizumab monotherapy for those patients with deficient DNA mismatch repair. | Recommended | The PBAC recommended the listing of pembrolizumab in combination with lenvatinib for the treatment of patients with advanced endometrial cancer who have disease progression following prior systemic therapy regardless of biomarker status. The PBAC considered that pembrolizumab plus lenvatinib provides superior efficacy, including improvements in overall survival, compared with chemotherapy. The PBAC noted this clear benefit was observed in all-comers in the key trial (KN775) regardless of biomarker status. The PBAC considered that pembrolizumab plus lenvatinib would be cost-effective at the cost per patient proposed by the sponsor.<br><br>In making this recommendation, the PBAC acknowledged the high unmet need for effective therapies to treat this condition.<br><br>While the submission had also proposed that pembrolizumab be used as monotherapy in patients with deficient DNA mismatch repair endometrial cancer, the PBAC recommended combination therapy (pembrolizumab plus lenvatinib) be listed for all patients regardless of biomarker status, consistent with the key randomised trial data. The PBAC considered that the incremental benefit of pembrolizumab monotherapy, which was based on a relatively small single arm study, had not been adequately quantified in the submission. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| 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>Category 2 submission<br>(Change to PBS listing) | Renal cell carcinoma | To request a Section 100 (Efficient Funding of Chemotherapy Program) Authority Required (STREAMLINED) listing, in combination with lenvatinib, for the first-line treatment of patients with advanced clear cell variant renal cell carcinoma. | Recommended | The PBAC recommended the listing of pembrolizumab for use in combination with lenvatinib (PEM+LEN) for the treatment of advanced (Stage IV) clear cell variant renal cell carcinoma (aRCC) in patients who are classified as intermediate or poor risk. The PBAC was satisfied that PEM+LEN was likely non-inferior to the nominated comparator, nivolumab plus ipilimumab (NIVO+IPI) in terms of overall survival and has a different, yet non-inferior, safety profile. The PBAC recommended listing on a cost-minimisation basis compared to NIVO+IPI, taking into account the likely duration of treatment for each therapy. The PBAC advised that PEM+LEN should join the existing risk sharing arrangement. The PBAC recommended potential flow on changes to the PBS restrictions for LEN to allow its use in aRCC and to nivolumab to align wording around the prognostic risk score assessment. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| 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>Early resolution submission<br>(Change to PBS listing) | Oesophageal carcinoma or HER-2-negative gastroesophageal junction adenocarcinoma | Resubmission to request listing a Section 100 (Efficient Funding of Chemotherapy Program) Authority Required (STREAMLINED) listing for the first-line treatment of locally advanced or metastatic oesophageal carcinoma or HER-2-negative gastroesophageal junction adenocarcinoma. | Deferred | The PBAC deferred making a recommendation to list pembrolizumab in combination with chemotherapy for the first-line treatment of advanced or metastatic gastro-oesophageal cancers as defined by the specific tumour types included in the approved TGA registered indications. The PBAC advised further discussions were required regarding appropriate restriction criteria, a cost-effective price and parameters for a risk sharing arrangement.<br><br><u>Sponsor Comment:</u><br>MSD is concerned with the decision to defer making a recommendation for pembrolizumab in combination with chemotherapy for the treatment of gastro-oesophageal cancers given MSD committed to the early resolution pathway in December 2021. MSD notes the broad listing approach in line with TGA indications under consideration will create complexities in developing an RSA that will be acceptable. |
| QUADRIVALENT INFLUENZA VACCINE (SURFACE ANTIGEN, INACTIVATED, CELL-BASED)<br><br>Injection 15 microgram in 0.5 mL needle-free pre-filled syringe<br>Injection 15 microgram in 0.5 mL pre-filled syringe with attached needle<br><br>Flucelvax® Quad<br><br>Seqirus (Australia) Pty Ltd<br><br>Category 2 submission<br>(New listing) | Prevention of influenza | To request National Immunisation Program listing for the prevention of influenza. | Not applicable | This submission was withdrawn. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| RABEPRAZOLE<br><br>Tablet containing rabeprazole sodium 20 mg (enteric coated)<br><br>Pariet®<br><br>Janssen-Cilag Pty Ltd<br><br>Committee secretariat submission (Change to PBS listing) | Complex gastro-oesophageal reflux disease<br>Scleroderma oesophagus<br>Gastro-oesophageal reflux disease<br>Peptic ulcer | To request a General Schedule Authority Required (STREAMLINED) listing of a new pack size (28 tablets per pack) under the same conditions as the currently listed 20 mg rabeprazole tablets (30 tablets per pack). | Recommended | The PBAC recommended the listing of rabeprazole 20 mg 28-tablet pack at an equivalent price per tablet to the currently listed product with a maximum quantity of 28 tablets, noting that the sponsor intends to delist the 30-tablet pack. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| RANIBIZUMAB<br><br>Solution for ocular implant 39.5 mg in 0.395 mL<br><br>Susvimo®<br><br>Roche Products Pty Ltd<br><br>Category 2 submission<br>(New PBS listing) | Neovascular (wet) age-related macular degeneration | To request a General Schedule Authority Required listing for the treatment of neovascular (wet) age-related macular degeneration responsive to prior anti-vascular endothelial growth factor (anti-VEGF) treatment. | Deferred | The PBAC deferred making a recommendation to list ranibizumab delivered via port delivery system (ranibizumab PDS), for the treatment of neovascular age-related macular degeneration (nAMD) in patients who have previously demonstrated a response to intravitreal anti-vascular endothelial growth factor (anti-VEGF) treatment. The PBAC considered that the clinical claim of noninferior effectiveness of ranibizumab PDS compared with intravitreal ranibizumab may be reasonable based on change from baseline in BCVA score, although there was an initial worsening in BCVA associated with the surgical procedure in the pivotal ARCHWAY trial and a small proportion of patients treated with ranibizumab PDS required supplementary treatment with intravitreal ranibizumab in addition to the 24-weekly refill-exchange procedures. The PBAC considered that the majority of the safety concerns associated with ranibizumab PDS were associated with the surgical procedure to implant the ocular device, and noting that the safety issues are being considered by the TGA, it considered that a positive Delegate's Overview would be required to clarify the overall safety profile of the drug. The PBAC was of a mind to recommend ranibizumab PDS, pending TGA advice and any advice from the MSAC about the procedures associated with the implanted PDS. A revised cost-minimisation approach and budget impact estimates would also be required.<br><br><u>Sponsor's Comment:</u><br>Roche looks forward to working with the PBAC to progress consideration of ranibizumab PDS (Susvimo®) and enable access for those patients who prefer an implanted device over intravitreal injections. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| RISANKIZUMAB<br><br>Injection 150 mg in 1 mL pre-filled syringe<br>Injection 150 mg in 1 mL pre-filled pen<br><br>Skyrizi®<br><br>AbbVie Pty Ltd<br><br>Category 2 submission<br>(New PBS listing) | Psoriatic arthritis | To request a General Schedule Authority Required (Written) listing for the treatment of severe psoriatic arthritis. | Recommended | The PBAC recommended the General Schedule, Authority Required (Written) listing of risankizumab (RIS) for the treatment of severe psoriatic arthritis (PsA), on a cost-minimisation basis with the least costly biological disease modifying anti-rheumatic drug (bDMARD) for this condition.<br><br>In making this recommendation, the PBAC accepted that any of the currently PBS listed bDMARDs for severe PsA could be an alternative therapy to RIS. The PBAC considered that RIS must be less expensive than the 'higher tier' bDMARDs to account for the lack of evidence to support non-inferiority to the higher tier medicines and could not be any more costly than any of the 'lower tier' bDMARDs currently listed on the PBS for this condition.<br><br>The Committee noted the flow-on changes to the administrative notes common to bDMARD listings to include RIS in the list of therapies in a PsA treatment cycle. |
| RISANKIZUMAB<br><br>Injection 150 mg in 1 mL pre-filled pen<br>Injection 150 mg in 1 mL pre-filled syringe<br>Injection 75 mg in 0.83 mL pre-filled syringe<br><br>Skyrizi®<br><br>AbbVie Pty Ltd<br><br>Category 3 submission<br>(Change to PBS listing) | Severe chronic plaque psoriasis | To request adding a grandfathering restriction to allow eligible patients enrolled in the risankizumab open-label extension trial (M15-997) to transition to PBS-subsidised risankizumab. | Recommended | The PBAC recommended extension of the listing of risankizumab 75 mg in 0.83 mL pre-filled syringe for the treatment of severe chronic plaque psoriasis to allow access for patients enrolled in the risankizumab open label extension trial (M15-997) to be grandfathered on to PBS-subsidised risankizumab. The PBAC considered that, should the recommended risankizumab 150 mg/mL PFP and 150 mg/mL PFS list before the end of the grandfathering period, these listings should include the same grandfather provisions. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| SACITUZUMAB GOVITECAN<br><br>Powder for injection 180 mg<br><br>Trodelvy®<br><br>Gilead Sciences Pty Limited<br><br>Early resolution submission<br>(New PBS listing) | Breast cancer | Resubmission to request a Section 100 (Efficient Funding of Chemotherapy Program) Authority Required (STREAMLINED) listing for the treatment of adult patients with unresectable, locally advanced or metastatic triple negative breast cancer, who have received two or more prior therapies, at least one of them in the locally advanced or metastatic setting. | Recommended | The PBAC recommended the listing of sacituzumab govitecan for the treatment of patients with unresectable locally advanced or metastatic triple negative breast cancer who have received at least two prior therapies. The PBAC considered that changes to the economic evaluation and financial estimates had sufficiently addressed its previous concerns.<br><br>The PBAC re-iterated its previous advice that sacituzumab govitecan provides superior efficacy compared with the current standard of care, notably an improvement in overall survival. The PBAC also acknowledged the high clinical need for effective therapies for patients with this condition, who have poorer survival outcomes than patients with other breast cancer subtypes. |
| SEBELIPASE ALFA<br><br>Solution concentrate for I.V. infusion 20 mg in 10 mL<br><br>Kanuma®<br><br>Alexion Pharmaceuticals Australasia Pty Ltd<br><br>Category 1 submission<br>(New PBS listing) | Infantile onset lysosomal acid lipase deficiency | To request a Section 100 (Highly Specialised Drugs Program) Authority Required (Written) listing for the treatment of infantile onset lysosomal acid lipase deficiency. | Not Recommended | The PBAC did not recommend the Section 100 (Highly Specialised Drugs Program) listing of sebelipase alfa for the treatment of infantile onset lysosomal acid lipase deficiency (LAL-D). The PBAC considered that, while sebelipase alfa was an effective treatment for infantile onset LAL-D, the incremental cost-effectiveness ratio for sebelipase alfa compared to best supportive care was extremely high and uncertain.<br><br><u>Sponsor Comment:</u><br>Alexion welcomes the PBAC's acknowledgment that sebelipase alfa is an effective treatment for infantile onset LAL-D, an ultra-rare disease. We will continue to pursue funding for this medicine where there are no alternative therapies available. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| SECUKINUMAB<br><br>Solution for injection 300 mg in 2 mL pre-filled pen<br>Solution for injection 300 mg in 2 mL pre-filled syringe<br><br>Cosentyx®<br><br>Novartis Pharmaceuticals Australia Pty Limited<br><br>Category 4 submission<br>(New PBS listing) | Non-radiographic axial spondyloarthritis<br>Severe active psoriatic arthritis<br>Severe psoriatic arthritis<br>Ankylosing spondylitis<br>Active ankylosing spondylitis<br>Severe chronic plaque psoriasis | To request General Schedule Authority Required (Written) listings of new forms of secukinumab under the same indications as the currently listed 150 mg secukinumab pre-filled pen and pre-filled syringe. | Recommended | The PBAC recommended General Schedule listings for two new forms of secukinumab injection (Cosentyx®): a 300 mg/2 mL pre-filled pen (PFP) and a 300 mg/2 mL pre-filled syringe (PFS) for severe psoriatic arthritis, severe chronic plaque psoriasis and ankylosing spondylitis, under the same circumstances as the current secukinumab 150 mg/mL PFP listing. |
| SELINEXOR<br><br>Tablet 20 mg<br><br>Xpovio®<br><br>Antengene (Aus) Pty Ltd<br><br>Standard re-entry submission<br>(New PBS listing) | Triple class refractory/penta-refractory multiple myeloma | Resubmission to request a Section 100 (Highly Specialised Drugs Program) Authority Required listing, for use in combination with dexamethasone, for the treatment of adult patients with relapsed and/or refractory multiple myeloma, who have received at least four prior therapies and whose disease is refractory to at least two proteasome inhibitors, at least two immunomodulatory agents, and an anti-CD38 monoclonal antibody. | Recommended | The PBAC recommended the listing of selinexor, for use in combination with dexamethasone (Sd), for the treatment of triple class refractory and penta-refractory multiple myeloma (TCR/PR MM). The PBAC considered that the clinical evidence for Sd was adequate to support listing in a small patient population with significant unmet need, and on this basis was satisfied that Sd provides, for some patients, a significant improvement in efficacy over salvage chemotherapy, represented by dexamethasone + cyclophosphamide + etoposide + cisplatin. The PBAC noted that the resubmission had addressed a number of its previous concerns with the economic model. The PBAC considered that the incremental cost effectiveness ratio was high at the proposed price and a price reduction would be required to ensure Sd is cost-effective in this population. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| SELINEXOR<br><br>Tablet 20 mg<br><br>Xpovio®<br><br>Antengene (Aus) Pty Ltd<br><br>Standard Re-entry submission (New PBS listing) | Relapsed and/or refractory multiple myeloma (RRMM) | Resubmission to request a Section 100 (Highly Specialised Drugs Program) Authority Required listing, for use in combination with bortezomib and dexamethasone, for the treatment of RRMM. | Not Recommended | The PBAC did not recommend the listing of selinexor, for use in combination with bortezomib and dexamethasone (SBd), for the treatment of RRMM in patients who have received at least one prior therapy.<br><br>The previous submission was considered in July 2021.<br><br><u>Comparator: carfilzomib plus dexamethasone (Cd).</u><br><br>The PBAC considered that Cd was a relevant comparator; however, noted that the multiple treatment options for RRMM and the changing treatment algorithm complicated the selection of the main comparator(s). The PBAC considered that Cd remained an important comparator.<br><br><u>Clinical claim: non-inferior effectiveness and safety compared with Cd.</u><br><br>The clinical claim was based on indirect treatment comparisons of SBd and Cd via Bd informed by two head-to-head trials: BOSTON (SBd vs Bd); and ENDEAVOR (Cd vs Bd). The resubmission presented updated data from BOSTON compared to the July 2021 submission.<br><br>The PBAC considered that the claim of non-inferior effectiveness was not adequately supported by the data. The PBAC noted the additional analyses of progression free survival provided by the resubmission, however considered that a lack of a statistically significant difference between SBd and Cd did not adequately establish non-inferiority. Secondly, the PBAC noted that a statistically significant improvement in overall survival was demonstrated for Cd versus Bd (in ENDEAVOR), but not for SBd versus Bd (in BOSTON). |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| | | | | The PBAC considered that the non-inferior safety claim was reasonable.<br><br><u>Economic claim: cost-minimisation versus Cd</u><br><br>The economic analysis was not informative, as it relied on an assumption of non-inferiority that was not adequately supported.<br><br>The PBAC considered that the utilisation estimates for SBd were uncertain, noting that the place in therapy for SBd and the impact on other RRMM regimens was unclear.<br><br><u>Sponsor Comment:</u><br>Antengene wishes to thank all of the healthcare professionals, patient organisations and patients for their support of our submission. We are disappointed with this outcome and will continue to work with the PBAC to provide access to selinexor in combination with bortezomib and dexamethasone. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| SEMAGLUTIDE<br><br>Injection 0.25 mg in 0.5 mL pre-filled single dose pen<br>Injection 0.5 mg in 0.5 mL pre-filled single dose pen<br>Injection 1.0 mg in 0.5 mL pre-filled single dose pen<br>Injection 1.7 mg in 0.75 mL pre-filled single dose pen<br>Injection 2.4 mg in 0.75 mL pre-filled single dose pen<br><br>Wegovy®<br><br>Novo Nordisk Pharmaceuticals Pty. Limited<br><br>Category 2 submission<br>(New PBS listing) | Obesity | To request a General Schedule Authority Required (STREAMLINED) listing for the treatment of obesity. | Not Recommended | The PBAC did not recommend the listing of semaglutide for the treatment of obesity. The PBAC recognised the high burden of disease in Australia, welcomed the input from a stakeholder forum prior to the meeting and noted the recently released *National Obesity Strategy 2022-2032*. The PBAC agreed with the submission that semaglutide 2.4 mg should be used as a later-line therapy, in conjunction with diet and exercise. However, the submission had poorly justified the population access it had requested (chronic use in patients with BMI ≥ 35 kg/m2 and at least one weight-related comorbidity but who do not have diabetes). In addition, although the trial data showed that semaglutide plus diet and exercise was superior to diet and exercise alone in terms of weight loss, HbA1c and other biomarkers and quality of life, these benefits were only demonstrated over the short term and whilst on treatment, and it was unlikely they would be fully realised in Australian practice without the intensive diet and exercise counselling co-administered in the trial program. Moreover, the submission's modelled reduction in comorbidities over a lifetime was highly uncertain given that no longer term data was presented in this submission. In addition, the PBAC noted that there were several issues with the economic model that limited its reliability, and that semaglutide was not cost-effective at the proposed price. Furthermore, the PBAC considered that pharmacotherapy was only one aspect of the public health response to obesity in Australia, but the proposed semaglutide PBS listing would require an extremely high investment (an average of >$1 billion annually over 6 years) with very uncertain implications for the PBS and broader health budget. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| | | | | Sponsor Comment:<br>Novo Nordisk looks forward to working with the PBAC to ensure timely access to Wegovy® (semaglutide) for the treatment of obesity, a disease impacting millions of Australians with no PBS reimbursed treatment options currently available. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| SOMAPACITAN<br><br>Injection 10 mg in 1.5 mL pre-filled pen<br><br>Sogroya®<br><br>Novo Nordisk Pharmaceuticals Pty. Limited<br><br>Category 2 submission<br>(New PBS listing) | Adult growth hormone deficiency | To request a Section 100 (Growth Hormone Program) Authority Required (Written) listing for the treatment of adult-onset growth hormone deficiency. | Recommended | The PBAC recommended the Section 100 (Growth Hormone Program) listing of somapacitan for the treatment of adults with growth hormone deficiency (AGHD) in patients aged 18 years and above, and those under 18 years of age with a mature skeleton. The PBAC's recommendation for listing was based on, among other matters, its assessment that the cost-effectiveness of somapacitan would be acceptable if it were cost-minimised to somatropin for the same indication. The PBAC advised that the equi-effective doses were somatropin 0.27 mg daily (1.89 mg weekly) and somapacitan 2.33 mg weekly, at the weekly dose relativity of 1 mg somatropin = 1.23 mg somapacitan.<br><br>The PBAC considered that patients who initiate on somatropin or somapacitan for the same indication do not need to meet the initial restriction criteria again for that indication if they have met the criteria on either somatropin or somapacitan. Additionally, the PBAC considered that the restrictions should include adult patients who have adult onset GHD, adult patients with documented childhood onset GHD and patients who have a mature skeleton but are under the age of 18 years. The PBAC recommended these changes flow-on to the PBS restrictions for somatropin for the same indication. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| SOMATROGON<br><br>Injection 24 mg in 1.2 mL pre-filled single-use pen<br>Injection 60 mg in 1.2 mL pre-filled single-use pen<br><br>Ngenla®<br><br>Pfizer Australia Pty Ltd<br><br>Category 2 submission<br>(New PBS listing) | Paediatric growth hormone deficiency | To request a Section 100 (Growth Hormone Program) Authority Required (Written) listing for the treatment of paediatric patients with growth hormone deficiency. | Recommended | The PBAC recommended the Section 100 (Growth Hormone Program) listing of somatrogon for the treatment of short stature associated with biochemical growth hormone deficiency (SSABGHD) and short stature and slow growth (SSSG) in patients who do not have a mature skeleton (i.e. who have a bone age of less than 13.5 years in females or less than 15.5 years in males). The PBAC's recommendation for listing was based on, among other matters, its assessment that the cost-effectiveness of somatrogon would be acceptable if it were cost-minimised to somatropin for the treatment of SSABGHD or SSSG. The PBAC advised that the equi-effective doses were 0.655 mg/kg/week of somatrogon and 0.237 mg/kg/week (0.034 mg/kg/day) of somatropin.<br><br>The PBAC considered that it is appropriate to allow switching between a daily dose regimen and a weekly dose regimen of somatropin and somatrogon respectively for the two above-mentioned indications, without having to be re-assessed where the patient has previously met the initial restriction criteria on either somatropin or somatrogon. Additionally, the PBAC considered that the restrictions should allow patients who do not have a mature skeleton to be eligible for somatrogon. The PBAC recommended these changes flow-on to the PBS restrictions of somatropin for the same indications. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| SOTORASIB<br><br>Tablet 120 mg<br><br>Lumakras®<br><br>Amgen Australia Pty Limited<br><br>Category 1 submission<br>(New PBS listing) | Non-small cell lung cancer (NSCLC) | To request a General Schedule Authority Required listing for the treatment of Kirsten rat sarcoma (KRAS) G12C variant non-squamous or not otherwise specified (NOS) stage IIIB (locally advanced) or Stage IV (metastatic) NSCLC in patients who have progressed on prior therapy. | Not Recommended | The PBAC decided not to recommend sotorasib for the treatment of patients with non-squamous or NOS Stage IIIB (locally advanced) or Stage IV (metastatic) NSCLC who harbour the KRAS G12C variant and who have progressed on prior therapy. The PBAC considered it was likely that sotorasib provided some clinical benefit over docetaxel but the magnitude of the benefit was highly uncertain. The PBAC considered the incremental cost-effectiveness ratio was uncertain and unacceptably high at the proposed price.<br><br><u>Sponsor Comment:</u><br>LUMAKRAS® (sotorasib) recently received provisional TGA approval for the treatment of adult patients with KRAS G12C-mutated locally advanced or metastatic non-small cell lung cancer (NSCLC) who have received at least one prior systemic therapy for advanced disease. Amgen will continue to work with the PBAC to secure reimbursement of sotorasib for eligible Australian lung cancer patients. |
| TEPOTINIB<br><br>Tablet 225 mg (as hydrochloride monohydrate)<br><br>Tepmetko®<br><br>Merck Healthcare Pty Ltd<br><br>Matters outstanding<br>(New PBS listing) | Non-small cell lung cancer (NSCLC) | To request a General Schedule Authority Required (STREAMLINED) listing for the treatment of patients with locally advanced or metastatic MET exon 14 skipping alterations-positive NSCLC. | Recommended | The PBAC recommended the listing of tepotinib for the treatment of patients with locally advanced (Stage IIIB) or metastatic (Stage IV) NSCLC who have evidence of a MET proto-oncogene, receptor tyrosine kinase (MET) gene alteration that causes skipping of exon 14 (METex14sk). |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| TRIENTINE<br><br>Tablet 150 mg (as tetrahydrochloride)<br><br>Cuprior®<br><br>Orphalan<br><br>Early re-entry submission (New PBS listing) | Wilson disease | Resubmission to request a General Schedule Authority Required listing for the treatment of patients with Wilson disease who are intolerant to penicillamine. | Not Recommended | The PBAC did not recommend trientine tetrahydrochloride (4HCl) for the treatment of patients with Wilson disease (WD) who are intolerant to penicillamine/D-penicillamine (DPA). The PBAC noted that the only changes in the resubmission were a small price reduction and a proposed risk sharing arrangement. The PBAC therefore considered that the economic analysis remained uninformative and the price differential compared to DPA was not justified, even with a second-line listing for trientine, given the clinical data was more consistent with non-inferiority to DPA.<br><br>The original submission was considered in November 2021.<br><br><u>Comparator: best supportive care (BSC)</u><br>The PBAC considered that the nominated comparator was reasonable.<br><br><u>Clinical claim: non-inferior effectiveness and superior safety compared with DPA and superior effectiveness and inferior safety compared with BSC</u><br>As no new clinical evidence was presented the PBAC reiterated its comments from November 2021.<br><br>Trientine 4HCl was non-inferior to DPA in terms of effectiveness and this was consistent with the accepted clinical approach and available guidelines. Trientine 4HCl was likely superior to DPA in terms of safety. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| | | | | Trientine 4HCl was superior to BSC in terms of effectiveness on the basis of chelation therapy being accepted as an effective and lifesaving treatment for WD. The claim that trientine 4HCl was inferior to BSC in terms of safety could not be assessed based on the evidence presented.<br><br><u>Economic claim: cost-utility versus BSC</u><br>As the only change to the economic analysis was the inclusion of the price reduction, the model remained uninformative.<br><br><u>Sponsor Comment:</u><br>The sponsor had no comment. |
| TRIENTINE<br><br>Capsule containing trientine dihydrochloride 250 mg (equivalent to 166.7 mg trientine)<br><br>Waymade®<br><br>Clinect Pty Ltd<br><br>Early re-entry<br>(New PBS listing) | Wilson disease | Resubmission to request a General Schedule Authority Required (Written) listing for the treatment of patients with Wilson disease who are intolerant to penicillamine. | Not Recommended | The PBAC did not recommend trientine dihydrochloride (2HCl) for the treatment of patients with Wilson disease (WD) who are intolerant to penicillamine/D-penicillamine (DPA). The PBAC noted that the only changes in the resubmission were a small price reduction, minor changes to the utilisation and financial impact estimates and a proposed risk sharing arrangement. The PBAC therefore considered that the economic analysis remained uninformative and the price differential compared to DPA was not justified, even with a second-line listing for trientine 2HCl, given the clinical data was more consistent with non-inferiority to DPA.<br><br>The original submission was considered in November 2021.<br><br><u>Comparator: no active treatment</u><br>The PBAC considered that the nominated comparator was reasonable. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| | | | | Clinical claim: superior effectiveness and safety compared with no active treatment<br>As no new clinical evidence was presented the PBAC reiterated its comments from November 2021.<br><br>Trientine 2HCl was superior to no active treatment in terms of effectiveness and safety on the basis of chelation therapy being accepted as an effective and lifesaving treatment for WD.<br><br>Economic claim: cost-utility versus no active treatment<br>As the only change to the economic analysis was the inclusion of the price reduction, the model remained uninformative.<br><br>Sponsor Comment:<br>The Sponsor continues to work with PBAC to achieve a way forward for this item. |
| TRIGLYCERIDES, MEDIUM CHAIN<br><br>Oral liquid 225 mL, 15 (K.Quik)<br><br>K.Quik®<br><br>Vitaflo Australia Pty Limited<br><br>Committee secretariat submission (Change to PBS listing) | Ketogenic diet | To request a General Schedule Authority Required (STREAMLINED) listing of a new brand to replace the currently listed Betaquik® brand. | Recommended | The PBAC recommended the listing of the new form of triglycerides, medium chain – oral liquid 225 mL, 15 (K.Quik) to replace the PBS-listed Betaquik. |

| | | | | |
|---|---|---|---|---|
| VERICIGUAT<br><br>Tablet 2.5 mg<br>Tablet 5 mg<br>Tablet 10 mg<br><br>Verquvo®<br><br>Bayer Australia Ltd<br><br>Category 2 submission<br>(New PBS listing) | Chronic heart failure | To request a General Schedule Authority Required (STREAMLINED) listing for the treatment of symptomatic (NYHA class II, III or IV) chronic heart failure in patients with a reduced ejection fraction (left ventricular ejection fraction less than 45%) and who are stabilised after a recent decompensation heart failure event requiring hospitalisation and/or intravenous diuretic therapy. | Not Recommended | The PBAC did not recommend vericiguat for the treatment of patients with symptomatic chronic heart failure who are stabilised after a recent decompensation event requiring hospitalisation and/or intravenous diuretic therapy and are on concomitant standard of care (SoC) therapies. The PBAC considered that vericiguat was associated with modest efficacy in a small group of high-risk patients. The PBAC advised that the proposed PBS restriction required revision to align more closely with these high-risk patients. Further, the PBAC considered the subgroup of patients included in the economic evaluation as a proxy for identifying stabilised patients was not an appropriate approach to the assessment of cost-effectiveness. The PBAC considered that the economic model should be revised to reflect the intention-to-treat population of the key trial and that the incremental cost-effectiveness ratio was unacceptably high and uncertain at the proposed price. The PBAC considered, therefore, that a price reduction would be required to achieve a cost-effective listing and the financial estimates would need to be revised, along with a risk sharing arrangement, to ensure PBS expenditure was restricted to this small group of patients.<br><br>The PBAC nominated the Early Re-entry re-submission pathway for this item.<br><br><u>Sponsor Comment:</u><br>While the PBAC's decision not to recommend vericiguat for the treatment of high-risk patients with symptomatic chronic heart failure is disappointing, Bayer welcomes the PBAC's consideration of the clinical unmet need for these high-risk patients and will continue to work with the PBAC to enable earliest possible access to vericiguat for these patients. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME | |
|---|---|---|---|---|
| ZANUBRUTINIB<br><br>Capsule 80 mg<br><br>Brukinsa®<br><br>BeiGene Aus Pty Ltd<br><br>Facilitated resolution submission (Change to recommended PBS listing) | Waldenstrom macroglobulinemia | Resubmission to request a General Schedule Authority Required listing for the treatment of adult patients with Waldenstrom macroglobulinemia (WM). | Recommended | The PBAC recommended the listing of zanubrutinib for the treatment of WM in treatment-naïve patients who are unsuitable for chemo-immunotherapy and in relapsed/refractory patients who have received at least one prior chemo-immunotherapy. The PBAC is satisfied that zanubrutinib provides, for some patients, a significant improvement in efficacy over rituximab monotherapy and bendamustine and rituximab in the treatment-naïve (TN) and relapsed/refractory (R/R) populations respectively.<br><br>The PBAC again acknowledged there was a high and urgent unmet need for effective treatments for WM on the PBS. The PBAC also recognised the sponsor's participation in the Facilitated Resolution Pathway workshop, and efforts to address outstanding issues raised at the July 2021 PBAC meeting and discussed at the workshop. In the context of limited clinical data for a rare disease, the PBAC considered that the resubmission's revised model was sufficiently reliable for decision making, and that the listing would be cost-effective at a lower incremental cost-effective ratio than proposed in the resubmission to account for the uncertainty associated with the modelled gain in overall survival. |

## PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
## MARCH 2022 PBAC MEETING

| DRUG NAME, FORM(S), STRENGTH(S) AND SPONSOR | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME |
|---|---|---|---|
| FLUOCINOLONE ACETONIDE<br>Intravitreal injection 190 micrograms<br>Iluvien®<br>Specialised Therapeutics Pharma Pty Ltd | Diabetic macular oedema | Review of positive PBAC recommendations not accepted by applicants | Recommendation was rescinded by the PBAC, noting that there was not a high clinical need for the product. |
| MEPOLIZUMAB<br>Injection 100 mg in 1 mL prefilled syringe<br>Nucala®<br>GlaxoSmithKline Australia Pty Ltd | Severe asthma | Review of positive PBAC recommendations not accepted by applicants | Recommendation was rescinded by the PBAC, noting that other forms of the same strength product were available on the PBS. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME |
|---|---|---|---|
| Correspondence from the Australian College of Nurse Practitioners (ACNP) regarding nurse prescribing for molnupiravir | SARS-CoV-2 | To consider whether it would be appropriate to amend the current PBS listing for molnupiravir for the treatment of SARS-CoV-2 infection to allow nurse practitioners to prescribe as a PBS benefit | The PBAC recommended amending the current PBS listing for molnupiravir for treatment of SARS-CoV-2 infection to allow nurse practitioners to prescribe as a PBS benefit and to verify the rapid antigen test required for eligibility. |
| Utilisation and cost-analysis of PBS-listed medicines for pulmonary arterial hypertension (PAH) report<br><br>AMBRISENTAN<br>BOSENTAN<br>MACITENTAN<br>EPOPROSTENOL ILOPROST<br>SELEXIPAG<br>SILDENAFIL (20mg, 90 tablets)<br>TADALAFIL (20mg, 56 tablets)<br>RIOCIGUAT<br>(all brands) | Medicines specifically used to treat PAH | For the PBAC to consider proposed PBS restrictions and the estimated cost to the PBS of extending PBS subsidy to dual therapy with endothelin receptor antagonist (ERA) and prostanoid medicines, first line for patients with World Health Organisation (WHO) Functional Class (FC) IV symptoms and second line for patients with WHO FC III symptoms. | The PBAC considered the findings of the 'Medicine utilisation and cost analysis of Pharmaceutical Benefits Scheme (PBS) listed medicines for PAH' report; the December 2021 cross-sectional analysis of the Pulmonary Arterial Hypertension Society of Australia and New Zealand (PHSANZ) patient registry data; proposed PBS restrictions for dual ERA + prostanoid therapy; February 2022 DUSC advice; and the sponsors' pre-subcommittee and pre-PBAC responses.<br><br>Overall, the PBAC accepted the key findings presented in the medicine utilisation and cost analysis and the patient registry analysis.<br><br>The PBAC noted DUSC advice that subsidising dual ERA (ambrisentan, bosentan, macitentan) + prostanoid (epoprostenol, iloprost) therapy first line for patients with WHO FC IV symptoms and second line for patients with WHO FC III symptoms was unlikely to cause a major change in PBS prostanoid utilisation.<br><br>The PBAC also noted the DUSC advice and PHSANZ input that subsidy of ERA + prostanoid dual therapy will likely result in those patients currently using ERA + phosphodiesterase-5 inhibitor (PDE-5i) + prostanoid triple therapy shifting from PBS subsidised PDE-5i + PBS prostanoid (and receiving the ERA via non-PBS sources) to PBS subsidised ERA + PBS subsidised prostanoid (and receiving the PDE-5i via a non-PBS source). DUSC was concerned that this could lead to potential inequities in access to PDE-5i medicines for these patients.<br><br>The PBAC noted DUSC advice that the additional cost of subsidising the PDE-5i for this group of patients is less than $1 million per year and would have a small effect on the overall net cost to the PBS. The PBAC recommended the subsidy of ERA + prostanoid dual therapy, first line for patients with PAH with WHO FC IV symptoms and second line for patients with PAH with WHO FC III symptoms.<br><br>The PBAC considered that the estimated total additional PBS expenditure for ERA + prostanoid dual therapy of $40.9 million (published prices) over the five years 2022-26 was likely at the upper limit and requested that revised estimates be prepared closer to the time of implementation on the PBS. The PBAC noted that Grandfather patients had been included in the financial estimates. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME |
|---|---|---|---|
| | | | The PBAC recommended the listing of ERA + inhaled/intravenous prostanoid + PDE-5i triple therapy for patients with PAH WHO FC IV symptoms, based on DUSC advice that the additional cost of less than $1 million per year to the PBS would address current inequities in access and further align PBS restrictions with contemporary clinical guidelines.<br><br>The PBAC accepted the proposed ERA + prostanoid dual PBS restrictions, noting that flow on changes to ERA, prostanoid and PDE-5i medicine PBS restrictions, including grandfather restrictions, will be required to implement the recommendation as intended for ERA + inhaled/intravenous prostanoid + PDE-5i triple therapy. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME |
|---|---|---|---|
| Cost-effectiveness review (CER) of sodium-glucose cotransporter-2 (SGLT2) inhibitor medicines<br><br>**DAPAGLIFLOZIN**<br><br>Tablet 10 mg<br>Forxiga®<br>AstraZeneca Pty Ltd<br><br>**DAPAGLIFLOZIN + METFORMIN**<br><br>Tablet 5 mg + 1 g<br>Tablet 10 mg + 500 mg<br>Tablet 10 mg + 1 g<br>Xigduo XR®<br>AstraZeneca Pty Ltd<br><br>**EMPAGLIFLOZIN**<br><br>Tablet 10 mg<br>Tablet 25 mg<br>Jardiance®<br>Boehringer Ingelheim Pty Ltd<br><br>**EMPAFGLIFLOZIN + METFORMIN**<br><br>Tablet 5 mg + 1 g<br>Tablet 12.5 mg + 1 g<br>Tablet 5 mg + 500 mg<br>Tablet 12.5 mg + 500 mg<br>Jardiamet®<br>Boehringer Ingelheim Pty Ltd | SGLT2 inhibitor class of medicines<br><br>Type 2 diabetes mellitus (T2DM) | To compare the cost-effectiveness of PBS listed SGLT2 inhibitors to sulfonylureas, as add-on therapy to metformin for the treatment of T2DM | In response to a request from a stakeholder, the PBAC requested the Department undertake a cost-effectiveness analysis comparing SGLT2 inhibitor medicines to sulfonylureas, as add-on therapy to metformin. The stakeholder requested the PBAC recommend changes to PBS restrictions for SGLT2 inhibitors to be consistent with recent evidence and clinical guidelines. Following consideration of the CER, the PBAC recommended the restrictions for empagliflozin and dapagliflozin could be expanded to allow earlier subsidised access to SGLT2 inhibitors in patients with T2DM as add-on therapy to metformin if they have established cardiovascular disease (CVD) or are at high risk of CVD, without the requirement to have a specific unmet glycaemic target. The PBAC also requested the Department progress pricing and risk sharing negotiations with the sponsors to achieve a financial cap on the additional cost to the PBS over the forward estimates. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME |
|---|---|---|---|
| Review of PBS Authority Required (Written) listings – Tranche 6 | Medicines for the treatment of rheumatology, dermatological and vasculitis conditions | To request that the Pharmaceutical Benefit Advisory Committee (PBAC) consider the Authority Required (Written) restriction level for PBS-listed medicines (Tranches 6) and recommend any required amendments. | The PBAC noted the key Review findings from the PBS Authority Required (Written) listings report, which included an analysis of PBS utilisation data for Tranche 6 medicines. The PBAC also noted the input provided by sponsors through submission of pre-subcommittee responses (PSCRs) on the written authority level of their Tranche 6 medicine(s).<br><br>The PBAC applied the following key criteria to assist in determining the requirement to maintain a written Authority level of restriction:<br>(1) Potential for use in a population in which the medicine is not cost-effective or where the PBAC has not determined the comparative effectiveness and cost; and (2) Potential for high cost per patient or high total cost to the health system and the Government's budget. The PBAC also considered the following factors: quality use of medicines (QUM), safety, and administrative burden.<br><br>Overall, the PBAC accepted the DUSC February 2022 advice on the need to amend or maintain the current written Authority level of each medicine and made the following recommendations: |
| 1. Adalimumab, certolizumab, etanercept, golimumab, infliximab, secukinumab | 1. Ankylosing spondylitis (AS) | | The PBAC noted that the market was stable and mature. Increases in costs in originator medicines (without biosimilars) were offset by the decrease in overall costs due to the uptake of biosimilar medicines for adalimumab, etanercept and infliximab. The PBAC considered that the recent listing of upadacitinib (1 October 2021) has the potential to grow the market.<br>The PBAC acknowledged the high administrative burden associated with the increasing volume of authority approvals.<br>The PBAC recommended an amendment to all initial treatment written authority levels of adalimumab, etanercept and infliximab biosimilar medicines for the treatment of AS to Authority Required (Telephone/Electronic), and to Authority Required (STREAMLINED) for first continuing treatment phases.<br>The PBAC did not recommend amendments to the authority requirements for adalimumab, etanercept and infliximab originator medicines, or for certolizumab, golimumab and secukinumab. The PBAC was mindful of supporting the differential between biosimilar and originator brands and of encouraging the prescribing of biosimilars where appropriate. |
| 2. Adalimumab, etanercept, tocilizumab | 2. Juvenile idiopathic arthritis (JIA) | | The PBAC noted that the JIA market was relatively small, stable, and mature, however there had been an increase in prevalent patients over the 2019/20-2020/21 period. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME |
|---|---|---|---|
| | | | The PBAC acknowledged that the overall volume of authority applications for JIA is small, but the associated administrative burden is high and distributed amongst a small prescriber group due to the current shortage of paediatric rheumatologists. The PBAC also recognised the administrative burden that falls to carers in securing specialist appointments to obtain prescriptions for treatment for patients with JIA.<br>Noting these concerns, the small market and equity of access for paediatric patients using biosimilar and originator medicines, the PBAC recommended an amendment to the initial treatment authority requirements for adalimumab, etanercept and tocilizumab (biosimilars and originators) from Authority Required (Written) to Authority Required (Telephone/Electronic) and, for first and subsequent continuing treatment from Authority Required (Written) to Authority Required (STREAMLINED). |
| 3. Tocilizumab | 3. Systemic juvenile idiopathic arthritis (sJIA) | | The PBAC acknowledged that while the overall volume of authority applications for sJIA is small, the associated administrative burden is high and distributed amongst a small prescriber group due to the current shortage of paediatric rheumatologists. The PBAC also recognised the administrative burden that falls to carers in securing specialist appointments to obtain prescriptions for treatment for patients with sJIA.<br>The PBAC recommended initial treatment authority requirements be amended from Authority Required (Written) to Authority Required (Telephone/Electronic) and continuing treatment be amended from Authority Required (Written) to Authority Required (STREAMLINED). |
| 4. Golimumab | 4. Non-radiographic axial spondyloarthritis (nr-axSpA) | | The PBAC noted that the market for nr-axSpA is immature and growing rapidly. The PBAC considered that recently listed medicines certolizumab (1 June 2020) and secukinumab (1 April 2021) may have impacted the utilisation of golimumab and that the pending listing of ixekizumab will further impact this market.<br>The PBAC recalled the 1 June 2020 amendments to the written authority requirements for the 'Initial treatment 2', 'Initial treatment 3' and continuing treatment restrictions to Authority Required (Telephone/Electronic).<br>The PBAC did not recommend an amendment to the authority requirements for golimumab for nr-axSpA at this time, noting the objective initial restriction criteria do not confer unnecessary administrative burden and ensure use in cost-effective populations. |
| 5. Adalimumab, certolizumab, etanercept, golimumab, infliximab, ixekizumab, secukinumab, tofacitinib, ustekinumab | 5. Psoriatic arthritis (PsA) | | The PBAC noted the PsA market was well established but not yet stable. The PBAC also noted the potential for further market growth with the recent PBS listing of new medicines that will not have biosimilars available in the short term. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME |
|---|---|---|---|
| | | | The PBAC did not recommend any changes to the current written authority requirements for medicines for PsA due to increasing utilisation, substantial increases in expenditure and the potential for use beyond the restriction within and outside of the indication. |
| 6. Abatacept, adalimumab, baricitinib, certolizumab, etanercept, golimumab, infliximab, rituximab, tocilizumab, tofacitinib | 6. Rheumatoid arthritis (RA) | | The PBAC noted that the market for RA was mature and moderately stable but that recent approvals (e.g., upadacitinib), had the potential for market disruption. Expenditure for infliximab, adalimumab, etanercept and rituximab had stabilised due to the availability of biosimilars and biosimilar uptake. The increased expenditure resulting from the introduction of JAK inhibitors (baricitinib, tofacitinib) has been offset by the PBS-listing of biosimilar medicines. However, the PBAC was mindful that expenditure on upadacitinib was not included in the review.<br>The PBAC noted the administrative burden for prescribers associated with the high volume of written authority applications and the objective initial and continuing treatment PBS restriction criteria for RA.<br>The PBAC recommended an amendment to the authority requirements for biosimilar medicines (adalimumab, etanercept, infliximab) for initial treatment 1, 2, 3 from Authority Required (Written) to Authority Required (Telephone/Electronic) and for first continuing treatment from Authority Required (Written) to Authority Required (STREAMLINED).<br>The PBAC considered that reducing the authority administrative burden for prescribers and patients may result in a preference for prescribing the older medicines where appropriate and a stabilisation of PBS expenditure.<br>The PBAC recommended an amendment to the current authority requirements for subsequent continuing treatment for adalimumab, etanercept and infliximab originator medicines from Authority Required (Written) to Authority Required (STREAMLINED). The PBAC also recommended an amendment to authority requirements for continuing treatment for abatacept, baricitinib, certolizumab, golimumab, tocilizumab and tofacitinib from Authority Required (Written) to First continuing treatment Authority Required (Written) and subsequent continuing treatment Authority Required (STREAMLINED). |
| 7. Adalimumab | 7. Hidradenitis suppurativa (HS) | | The PBAC noted that the market is not yet stable and that there has been a recent increase in the incident patient population and progressive increases in the treated prevalent patient population.<br>The PBAC did not recommend an amendment to the authority requirements for adalimumab, due to the risk of use beyond the restriction to a broad range of off-label dermatology indications. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME |
|---|---|---|---|
| 8. Adalimumab, etanercept, guselkumab, infliximab, ixekizumab, secukinumab, tildrakizumab, ustekinumab | 8. Severe chronic plaque psoriasis (adult) | | The PBAC noted the market is mature but not yet stable, with the introduction of newer biologic medicines coinciding with a progressive increase in treated prevalent patients. PBS expenditure on CPP has continued to grow over the Review period despite the availability of adalimumab, etanercept and infliximab biosimilars.<br>The PBAC did not recommend an amendment to the authority requirements for any medicines for CPP (adult) due to the market instability, growth in utilisation, growth in expenditure, the risk of use beyond the restriction and the financial risk to Government. |
| 9. Etanercept | 9. Severe chronic plaque psoriasis (juvenile) | | The PBAC noted the CPP (juvenile) market was small and mature. The PBAC also noted the potential for use of etanercept beyond the PBS restriction in a broad range of off-label indications but considered the risk low due to the distinct morphologic characteristics of CPP.<br>The PBAC recognised the need to reduce the administrative burden for biologics with paediatric indications for prescribers and the administrative burden that falls to carers in securing specialist appointments to obtain prescriptions for treatment for patients with CPP (juvenile). The PBAC considered an amendment to authority requirements would be appropriate to assist in ensuring timely access to treatment for patients.<br>The PBAC recommended initial treatment authority requirements for etanercept be amended from Authority Required (Written) to Authority Required (Telephone/Electronic) and continuing treatment be amended from Authority Required (Written) to Authority Required (STREAMLINED). |
| 10. Rituximab | 10.Granulomatosis (Vasculitis)<br>Granulomatosis with polyangiitis (GPA)<br>Granulomatosis with microscopic polyangiitis (MPA) | | The PBAC did not make a recommendation given pending implementation of its September 2021 recommendation for an unrestricted benefit listing for rituximab. |
| 11. Naltrexone, acamprosate | 11. Alcohol use disorder | | The PBAC noted the market was mature, however over the 2019/20-2020/21 period there has been an increase in incident patient utilisation and growth in the prevalent patient population. The PBAC considered that this may be related to an increase in alcohol consumption during the COVID-19 pandemic.<br>The PBAC noted that PBS expenditure on these medicines is increasing and is driven by naltrexone. |

| DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION | DRUG TYPE AND USE | LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION | PBAC OUTCOME |
|---|---|---|---|
| | | | The PBAC agreed that the authority requirements between acamprosate and naltrexone should be aligned and that this may reduce barriers to access to treatment for at risk populations.<br>The PBAC recommended an amendment to the current authority restriction for naltrexone for the treatment of alcohol use disorder from Authority Required (Telephone/Electronic) to Authority Required (STREAMLINED) and that the restriction's clinical criterion be amended to:<br>"The treatment must be part of a comprehensive treatment program with the goal of maintaining abstinence or controlled consumption". |

**Version 2**
Amendment
1. Added entry for ranibizumab (Susvimo®)

**Version 3**
Amendment
1. New text for Apremilast outcome.
2. New outcome for Beclometasone with Formoterol and Glycopyrronium.

**Version 4**
Amendment
1. New outcome for ranibizumab (Susvimo®)

**Version 5**
Amendment
1. Updated form for trientine dihydrochloride (Waymade®)
2. Updated drug name for diphtheria, tetanus, pertussis, hepatitis B, poliomyelitis and Haemophilus influenzae type b conjugate vaccine (DTPa-HB-IPV-Hib)

**Version 6**
Amendment
1. Outcome of Beclometasone with Formoterol and Glycopyrronium (Trimbow®) has been moved to the Recommendations made out-of-session by the PBAC meeting between meetings webpage to reflect it was considered out-of-session.

**PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES**
**MARCH 2022 PBAC MEETING**

**Submission category types**

| | |
|---|---|
| **Category 1** | A request for PBS or NIP listing of one or more of the following:<br>• A first in class medicine or vaccine, and/or a medicine or vaccine for a new population. OR<br>• A drug with a codependent technology that requires an integrated codependent submission to the PBAC and MSAC. OR<br>• A drug or designated vaccine with a TGA Provisional determination related to the proposed population. |
| **Category 2** | A request for PBS or NIP listing of a new medicine or new vaccine, a new indication of a currently listed medicine or vaccine, or to make material changes to a currently listed indication and do not meet the criteria for a Category 1 submission. |
| **Category 3** | Requests to change existing listings that do not change the population or cost-effectiveness of the medicine or vaccine that do not meet the criteria for a Category 4 submission. |
| **Category 4** | A request for one or more of the following:<br>• Listing of a new pharmaceutical item of a listed medicine.<br>• Consideration as an exempt item (Exempt item as per subsection 84AH of the *National Health Act 1953*).<br>• Including a listed medicine on the prescriber bag, or varying an existing prescriber bag listing.<br>• A change/new manner of administration of a listed medicine.<br>• A change to the maximum quantity and/or number of repeats of a listed medicine.<br>• A change or addition to the prescriber type(s) of a listed medicine. |
| **Committee Secretariat** | Application is not in Categories 1, 2, 3 or 4 and requests for one or more of the following:<br>• New or varied listed drugs, medicinal preparations and designated vaccines that pose no greater risk<br>• Pharmaceutical benefits that can no longer be supplied early<br>• New brand of glucose indicator pharmaceutical item. |

**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:<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 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:<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. |