PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
ABIRATERONE AND METHYLPREDNISOLONE Pack containing 120 tablets abiraterone (as acetate) 125 mg and 30 tablets methylprednisolone 4 mg Yonsa Mpred® Sun Pharma ANZ Pty Ltd Category 2 submission (Change to existing listing)	Metastatic hormone sensitive prostate cancer	To request a General Schedule Authority Required (Telephone/Online) listing for the treatment of patients with metastatic hormone sensitive prostate cancer.	Recommended	The PBAC recommended the listing of the composite pack of abiraterone acetate with 30 methylprednisolone (SAA+MPRED) tablets for the treatment of metastatic hormone sensitive prostate cancer on a cost-minimisation approach (CMA) versus apalutamide. The PBAC considered that SAA+MPRED, in combination with androgen deprivation therapy (ADT), was non-inferior to apalutamide plus ADT in terms of efficacy and although it was inferior in terms of safety, that the cost-offsets applied in the CMA were reasonable. The PBAC advised that SAA+MPRED should join the existing risk sharing arrangement for novel hormonal agents in this setting. The PBAC noted flow - on restriction changes to include SAA+MPRED in the administration note.
ACALABRUTINIB Tablet 100 mg Calquence® AstraZeneca Pty Ltd Standard re-entry submission (Change to existing listing)	Chronic lymphocytic leukaemia (CLL) or small lymphocytic lymphoma (SLL)	Resubmission to request a General Schedule Authority Required (Telephone/Online) listing, for use as monotherapy or in combination with obinutuzumab, for the treatment of previously untreated CLL or SLL.	Recommended	The PBAC recommended extending the listing of acalabrutinib (ACA) to include the treatment of patients with previously untreated CLL/SLL. The PBAC considered the nominated comparator of venetoclax plus obinutuzumab (VTX+OBIN) was appropriate. The PBAC's consideration was based on, among other matters, its assessment as described above, that the cost-effectiveness of ACA as monotherapy or in combination with obinutuzumab (OBIN) would be acceptable if it were cost-minimised to VTX+OBIN, and zanubrutinib (ZANU) if PBS-listed for previously untreated patients. The PBAC considered a risk sharing arrangement (RSA) would be required to manage the risks of the duration of treatment with ACA being longer than modelled and earlier use in patients not meeting the International Workshop on CLL criteria for initiating treatment. The PBAC advised that ACA should join the ZANU RSA should ZANU have progressed to PBS listing for previously untreated CLL/SLL. The PBAC noted flow-on restriction changes would be required for OBIN.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
ADALIMUMAB Injection 40 mg in 0.4 mL pre-filled pen Injection 40 mg in 0.4 mL pre-filled syringe Injection 80 mg in 0.8 mL pre-filled syringe Ardalicip® Cipla Australia Pty Ltd Category 3 submission (New listing)	Severe Crohn disease Moderate to severe ulcerative colitis Severe active juvenile idiopathic arthritis Complex refractory fistulising Crohn disease Severe active rheumatoid arthritis Severe psoriatic arthritis Ankylosing spondylitis Severe chronic plaque psoriasis Moderate to severe hidradenitis suppurativa	To request listing of an adalimumab biosimilar under the same conditions as its reference biologic.	Recommended	The PBAC recommended the Authority Required listing of adalimumab (Ardalicip®) in the form of 40 mg in 0.4 mL pre-filled syringe (PFS) and pre-filled pen (PFP) and 80 mg in 0.8 mL PFS as biosimilar brands of Humira® on the General Schedule and Section 100 (Highly Specialised Drug Program). The PBAC's recommendation for listing was based on, among other matters, its assessment that the cost - effectiveness of Ardalicip® PFP and PFS would be acceptable if it were cost minimised to the lowest cost PBS-listed adalimumab brand. The PBAC advised the equi-effective doses to be 1 mg of Ardalicip® = 1 mg of Humira® and all other biosimilar brands and formulations of adalimumab. The PBAC advised that Humira® and Ardalicip® PFS should be treated as equivalent to each other; and Humira® and Ardalicip® PFP should be treated as equivalent to each other for the purposes of substitution. The PBAC advised that 40 mg in 0.4 mL Ardalicip® PFS and 40 mg in 0.8 mL Amgevita®, Hadlima®, Hyrimoz® and Idacio® PFS should be treated as equivalent to each other for the purpose of substitution; 40 mg in 0.4 mL Ardalicip® PFP and 40 mg in 0.8 mL Amgevita®, Hadlima®, Hyrimoz® and Idacio® PFP should be treated as equivalent to each other for the purpose of substitution. The PBAC advised that Ardalicip® PFP should not be considered equivalent for the purposes of substitution with any adalimumab PFS, consistent with its previous considerations of adalimumab.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
ATOGEPANT Tablet 60 mg Aquipta® Allergan Australia Pty Limited Category 2 submission (New listing)	Prophylaxis of migraine	To request a General Schedule Authority Required (STREAMLINED) listing for the prophylaxis of high frequency episodic and chronic migraine.	Not Recommended	The PBAC did not recommend atogepant for use as prophylaxis in adult patients with chronic migraine or high frequency episodic migraine who have experienced an inadequate response, intolerance or a contraindication to at least three prophylactic migraine medications. The PBAC acknowledged that there was a need for an oral prophylactic migraine treatment but considered that the indirect treatment comparisons presented did not establish that atogepant was non-inferior in terms of effectiveness compared to the nominated comparators, galcanezumab and fremanezumab. The PBAC considered that the utilisation estimates were not adequately supported and that the structure of the risk sharing arrangement as proposed in the submission were poorly justified. <u>Sponsor's Comment:</u> AbbVie is disappointed with this outcome and is currently reviewing next steps.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
AVACOPAN Capsule 10 mg Tavneos® Vifor Pharma Pty Limited Category 1 submission (New listing)	Severe active granulomatosis with polyangiitis (GPA) and severe active microscopic polyangiitis (MPA)	To request a General Schedule Authority Required (STREAMLINED) listing for the treatment of severe active GPA and severe active MPA in combination with rituximab or cyclophosphamide/azathioprine.	Not Recommended	The PBAC did not recommend the listing for avacopan for the treatment of severe active GPA or severe active MPA in combination with rituximab or cyclophosphamide/azathioprine. The PBAC acknowledged there was a clinical need for treatments that can be used to reduce exposure to glucocorticoids (GCs) in this condition. The PBAC considered the key clinical data from the ADVOCATE trial suggested the magnitude of benefit that avacopan and standard of care (SOC) may provide in induction therapy compared to prednisolone and SOC was limited to a potential reduction in GC use with no significant benefit in remission at 26 weeks. In addition, the Committee considered the clinical evidence provided was inadequate to assess remission at 52 weeks and did not support comparative assessment of use as maintenance therapy. The PBAC considered the economic model was unreliable due to the uncertainties underpinning clinical data. Additional optimistic assumptions and inputs meant the PBAC considered the resulting incremental cost-effectiveness ratio was likely underestimated and highly uncertain. <u>Sponsor's Comment:</u> The sponsor had no comment.

DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION	DRUG TYPE AND USE	LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION	PBAC OUTCOME	
BUDESONIDE Tablet 500 micrograms (orally disintegrating) Tablet 1 mg (orally disintegrating) Jorveza® Dr Falk Pharma Australia Pty Ltd Matters outstanding (Change to PBS listing)	Eosinophilic oesophagitis (EoE)	To request PBAC advice regarding the removal of the histological assessment to determine eligibility for continuing treatment as deferred at the March 2023 PBAC meeting	Recommended	The PBAC recommended extending the timeframe for a histological assessment (endoscopy) following initiation of treatment to determine treatment response and eligibility for continuing treatment with budesonide orally disintegrating tablets (BOT) 500 micrograms and 1 mg for EoE. The PBAC considered that the timeframe could be extended from 8 - 10 weeks to within 12 months of starting treatment. The PBAC recommended patients could continue to access BOT through the PBS until they have the repeat endoscopy, subject to advice provided by their treating clinician. The PBAC recalled the concerns raised in the March 2023 submission that clinicians reported challenges for clinicians and patients in obtaining a follow-up endoscopy within the 8 - 10 week timeframe. The PBAC noted advice received from the Gastroenterological Society of Australia (GESA) that the current requirement for a second endoscopy within 8 - 10 weeks was difficult to meet, but a histological follow-up is still best practice. The PBAC noted GESA's suggestion to extend the time for a follow-up endoscopy. The PBAC also noted advice from GESA that BOT is well tolerated and has a favourable adverse effect profile and considered the risk to patients in extending the time for a follow-up endoscopy would be low. The PBAC noted the findings from the utilisation review of BOT by the Drug Utilisation Sub-Committee (DUSC) and the projected use of BOT for 12 months since initial listing. The PBAC noted the number of patients accessing continuing treatment was lower than predicted. The PBAC asked DUSC to continue to monitor the use of BOT and undertake a repeat review of its utilisation in 12 months' time.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
CABOTEGRAVIR Suspension for injection 600 mg in 3 mL Apretude® ViiV Healthcare Pty Ltd Category 2 submission (New listing)	Pre-exposure prophylaxis for human immunodeficiency virus (HIV) infection	To request a General Schedule Authority Required (STREAMLINED) listing for use as pre-exposure prophylaxis (PrEP) for HIV infection in persons in whom tenofovir/emtricitabine (TD/FTC) is contraindicated.	Deferred	The PBAC deferred making a recommendation for the General Schedule listing of cabotegravir long-acting injection (CAB-LA) for HIV PrEP to allow further deliberation on the population who should be eligible for CAB-LA as PrEP and the associated PBS restrictions. The PBAC noted the submission requested a listing for CAB-LA as PrEP only for people who are contraindicated or intolerant to, or were repeatedly non-adherent to oral TD/FTC to the extent efficacy and safety were compromised. The PBAC did, however, consider the clinical need for CAB-LA as a long-acting option arises from a diverse range of motivations and needs and was of the view that the PBS restrictions required further consideration and discussion with the Sponsor (ViiV Healthcare Pty Ltd) to achieve an equitable and cost - effective listing for CAB-LA. <u>Sponsor's Comment:</u> ViiV Healthcare welcomes the PBAC's recognition that CAB - LA PrEP is an important option for people who cannot use oral PrEP in an effective manner and that an additional PrEP regimen could increase uptake of PrEP in general and help meet Australian health policy goals. ViiV Healthcare strongly believes the evidence presented demonstrates superior efficacy in the proposed population and will work with the PBAC to progress to a recommendation at the earliest opportunity.

DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION	DRUG TYPE AND USE	LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION	PBAC OUTCOME	
CALCIPOTRIOL WITH BETAMETHASONE Foam containing calcipotriol 50 micrograms with betamethasone 500 micrograms (as dipropionate) per g, 60 g Enstilar® LEO Pharma Pty Ltd Category 3 submission (Change to existing listing)	Chronic stable plaque type psoriasis vulgaris	To request a General Schedule Authority Required (STREAMLINED) listing with an increased maximum quantity of two and maximum repeat of one.	Not Recommended	The PBAC did not recommend increasing the maximum quantity of calcipotriol 0.005% with betamethasone (as dipropionate) 0.05% foam. The PBAC considered that the current Authority provisions in place for prescribers to request larger quantities are sufficient. The PBAC recalled its previous consideration for calcipotriol 0.005% with betamethasone (as dipropionate) 0.05% gel (Daivobet®) at its March 2018 meeting and noted that no further evidence was provided regarding the risk of toxicity associated with increased access to larger quantities of calcipotriol. The PBAC recommended adding an administrative note to the existing listing to clarify the existing provision for prescribers to request an increased quantity. The PBAC noted restriction flow-on changes to calcipotriol 0.005% + betamethasone (as dipropionate) 0.05% ointment. <u>Sponsor's Comment:</u> LEO Pharma is disappointed that the PBAC has not recommended additional PBS listings with a higher maximum quantity for treatment of patients with chronic stable plaque type psoriasis vulgaris affecting a larger body surface area.
DAPAGLIFLOZIN Tablet 10 mg Forxiga® AstraZeneca Pty Ltd Category 2 submission (Change to existing listing)	Chronic heart failure (HF)	To request a General Schedule Authority Required (STREAMLINED) listing for patients with chronic heart failure with preserved ejection fraction (HFpEF).	Recommended	The PBAC recommended extending the listing of dapagliflozin to include the treatment of patients with chronic HF with a left ventricular ejection fraction (LVEF) greater than 40%. The PBAC considered that dapagliflozin was non-inferior to the main comparator, empagliflozin in terms of comparative effectiveness and safety. The PBAC's recommendation for listing was based on its assessment that the cost-effectiveness of dapagliflozin would be acceptable if it was cost - minimised against empagliflozin, which was recommended for listing for this indication in December 2022. The PBAC noted flow-on changes to the listing of empagliflozin to update criterion "Patient must be symptomatic with NYHA classes II, III or IV prior to initiating treatment with this drug" for this indication.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
DAPRODUSTAT Tablet 1 mg Tablet 2 mg Tablet 4 mg Tablet 6 mg Tablet 8 mg Jesduvroq® GlaxoSmithKline Australia Pty Ltd Category 2 submission (New listing) WITHDRAWN	Anaemia associated with chronic kidney disease	To request a Section 100 (Highly Specialised Drugs Program) Authority Required (STREAMLINED) listing for the treatment of anaemia associated with chronic kidney disease.	Not Applicable	This item was withdrawn.
DAUNORUBICIN WITH CYTARABINE Powder for I.V infusion containing daunorubicin 44 mg and cytarabine 100 mg Vyxeos® Jazz Pharmaceuticals ANZ Pty Ltd Category 1 submission (New listing)	Acute myeloid leukaemia	To request a Section 100 (Efficient Funding of Chemotherapy Program) Authority Required (Telephone/Online) listing for the treatment of therapy-related acute myeloid leukaemia (t-AML) and acute myeloid leukaemia with myelodysplasia-related changes (AML - MRC).	Not Recommended	The PBAC did not recommend liposomal daunorubicin and cytarabine for the treatment of t-AML or AML-MRC. The PBAC considered that liposomal daunorubicin and cytarabine had improved efficacy over the nominated comparator, idarubicin and cytarabine, in terms of overall survival; however, considered that the incremental cost-effectiveness ratio was high and uncertain. The PBAC also considered that the estimated utilisation and financial impact estimates were uncertain. The PBAC nominated the early re-entry pathway for this item. <u>Sponsor's Comment:</u> Jazz appreciates the opportunity to pursue an early re - entry submission to expedite patient access to Vyxeos®.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
DUPILUMAB Injection 200 mg in 1.14 mL single dose pre-filled syringe Injection 300 mg in 2 mL single dose pre-filled syringe Dupixent® Sanofi-Aventis Australia Pty Ltd Category 3 submission (Other matters)	Chronic severe atopic dermatitis (AD)	To request the PBAC consider the previously estimated utilisation for chronic severe AD.	Advice Provided	The PBAC advised that it would be reasonable for the current risk sharing arrangement (RSA) financial caps for dupilumab (and upadacitinib), for the treatment of severe AD in patients aged 12 years and older, to be increased for the remaining years of the arrangement, to account for patients with severe AD of the hands and/or face. In providing this advice, the PBAC noted that such use was not accounted for in the original RSA caps, however, given the apparent quality of life impacts of disease affecting the hands and/or face appear similar to that for the whole body, considered that use in these patients is likely to be cost-effective. The PBAC considered the submission's other proposed changes to the financial estimates (increasing the proportion of patients inadequately controlled on topical corticosteroids and increasing the uptake rates) to be overestimated and highly uncertain. The PBAC considered that the submission did not provide sufficient justification in relation to changing these assumptions and therefore did not support these amendments.
DURVALUMAB Solution concentrate for I.V. infusion 120 mg in 2.4 mL vial Solution concentrate for I.V. infusion 500 mg in 10 mL vial Imfinzi® AstraZeneca Pty Ltd Early re-entry submission (Change to existing listing)	Biliary tract cancer	Resubmission to request a Section 100 (Efficient Funding of Chemotherapy Program) Authority Required (STREAMLINED) listing for the treatment of advanced biliary tract cancer.	Recommended	The PBAC recommended the listing of durvalumab for the treatment of advanced biliary tract cancer. The PBAC considered that durvalumab in combination with chemotherapy provides a moderate improvement in overall survival and a small improvement in progression free survival compared to chemotherapy alone. The PBAC considered that the amendments made in the resubmission, including changes to the economic model and a reduced price had sufficiently addressed the Committee's previous concerns. The PBAC considered the utilisation remained overestimated and advised assumptions regarding uptake rate, proportion of patients with performance status 0 or 1 and proportion of patients with liver cancer who have intrahepatic cholangiocarcinoma should be revised.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
ENOXAPARIN Injection containing enoxaparin sodium 20 mg (2,000 I.U. anti-Xa) in 0.2 mL pre filled syringe Injection containing enoxaparin sodium 40 mg (4,000 I.U. anti-Xa) in 0.4 mL pre filled syringe Injection containing enoxaparin sodium 60 mg (6,000 I.U. anti-Xa) in 0.6 mL pre filled syringe Injection containing enoxaparin sodium 80 mg (8,000 I.U. anti-Xa) in 0.8 mL pre filled syringe Injection containing enoxaparin sodium 100 mg (10,000 I.U. anti-Xa) in 1 mL pre filled syringe Injection containing enoxaparin sodium 120 mg (12,000 I.U. anti-Xa) in 0.8 mL pre filled syringe Injection containing enoxaparin sodium 150 mg (15,000 I.U. anti-Xa) in 1 mL pre filled syringe Exarane™ Exarane Forte™ Juno Pharmaceuticals Pty Ltd Category 3 submission (New listing)	Thrombo-embolic disorders	To request listing of an enoxaparin biosimilar under the same conditions as its reference biologic, and to request a General Schedule Restricted Benefit listing for haemodialysis and a General Schedule unrestricted listing of two new strengths under the same conditions as the currently listed forms.	Recommended	The PBAC recommended the listing of biosimilar brand of enoxaparin (Exarane™ and Exarane Forte™) on the General Schedule on a cost-minimisation basis under the same circumstances as the PBS-listed reference biologic Clexane Safety-Lock®. The PBAC advised that the listing of Exarane™ be based on equi-effective doses of each corresponding PBS listed formulation of Clexane Safety-Lock® where 1 mg Exarane™/Exarane Forte™ = 1 mg Clexane Safety-Lock®. The PBAC also recommended the listing of two new strengths of enoxaparin, 120 mg in 0.8 mL and 150 mg in 1 mL (Exarane Forte™) and on a cost-minimisation basis with the least costly alternative presentation on a per milligram basis. The PBAC advised that the addition of an administrative note to encourage the uptake of biosimilar prescribing for treatment naïve patients would be appropriate, in accordance with the Government's policy to encourage the use of biosimilar medicines. The PBAC noted that it is not possible to implement the other biosimilar uptake driver of a less restrictive Authority type listing in this instance. The PBAC advised that Exarane™, Exarane Forte™ and Clexane Safety-Lock® should be considered equivalent for the purposes of substitution (i.e. 'a' flagged).

DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION	DRUG TYPE AND USE	LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION	PBAC OUTCOME	
ESKETAMINE Nasal spray solution 28 mg in 0.2 mL Spravato® Janssen-Cilag Pty Ltd Standard re-entry submission (New listing)	Treatment resistant depression (TRD)	Resubmission to request a Section 100 (Highly Specialised Drug Program) Authority Required (Telephone/Online) listing for the treatment of patients with TRD.	Not Recommended	The PBAC did not recommend the PBS listing of esketamine for the treatment of TRD. The PBAC noted, based on the esketamine stakeholder meeting held in February 2023 (outcome statement available here), it was proposed that a treatment course with esketamine be limited to a maximum of 12 months duration. The PBAC noted this was the key change in the resubmission which flowed into the economic and financial models. The PBAC acknowledged the input from patients, clinicians and organisations, many of whom highlighted the severe impacts of TRD on everyday life and described how esketamine (and ketamine) treatment had been transformative and restored hope in their lives. The previous submissions were considered in July 2021 and July 2022. Proposed restriction: The proposed restriction criteria limited esketamine to a maximum duration of 12 months per episode as supported by the stakeholder meeting. The PBAC considered there was a number of uncertainties associated with this, including how episodic treatment would be managed, the impact of ceasing treatment in responders and how to implement use beyond 12 months for some patients. The PBAC agreed with the input from the stakeholder meeting that it was appropriate that some patients who responded to esketamine in a depressive episode would use it again if they relapsed or experienced a new depressive episode. The PBAC considered that implementing an appropriate pathway for retreatment would require further consideration. Comparator: Placebo (+ a new oral antidepressant (OAD): The PBAC recalled its previous view that the nominated comparator of a newly initiated OAD alone was reasonable. The PBAC noted no new clinical evidence was provided in the resubmission. The PBAC recalled its previous view that the claim of superior comparative effectiveness may be reasonable, although the magnitude and clinical

DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION	DRUG TYPE AND USE	LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION	PBAC OUTCOME	
				importance of the observed benefits was uncertain. The PBAC noted the inclusion of a 12 month treatment cap per episode increased the uncertainty regarding the magnitude of clinical benefit that would be observed in clinical practice. Economic claim: Cost-utility versus placebo: The PBAC noted the economic model included a single course of treatment however, the model time horizon of 5 years was retained. The PBAC considered that while it was uncertain how many patients would receive more than one course of treatment over a 5 year period, it was likely to be a high proportion of patients. The PBAC considered it was unlikely that esketamine would be cost-effective if additional courses of treatment were required within the 5 year period. Utilisation of esketamine: The PBAC considered the uptake of esketamine in new patients was likely overestimated and while the uptake in subsequent episodes was uncertain, considered it was likely underestimated. The PBAC considered the financial estimates were overestimated due to optimistic assumptions regarding the uptake of esketamine in the context of likely barriers to access. Sponsor's Comment: Janssen is disappointed that the PBAC did not recommend esketamine but welcomes the PBAC's recognition of the need for treatment options for TRD, their understanding of the severe impact of TRD on daily life, and their acceptance of the clinically meaningful benefits of esketamine. Janssen believes in the benefit that esketamine provides patients and hopes to have this treatment available through the PBS. Janssen will consider how we can resolve the remaining uncertainties so that Australian patients can access esketamine in a timely way.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
FOSLEVODOPA WITH FOSCARBIDOPA Solution for subcutaneous infusion foslevodopa 2400 mg with foscarbidopa 120 mg in 10 mL Vyalev® Abbvie Pty Ltd Category 2 submission (New listing)	Advanced Parkinson's Disease	To request a General Schedule and Section 100 (Highly Specialised Drugs Program) Authority Required (STEAMLINED) listing for the treatment of advanced Parkinson's disease with severe disabling motor fluctuations not adequately controlled by oral therapy.	Not Applicable	This item is to be considered at a future PBAC meeting.
IXEKIZUMAB Injection 80 mg in 1 mL single dose pre-filled pen Taltz® Eli Lilly Australia Pty Ltd Category 4 submission (Other matters)	Non-radiographic axial spondyloarthritis (nr-AxSpA)	To request the PBAC consider listing ixekizumab for the treatment of nr-AxSpA in a pack size of two injections.	Not Recommended	The PBAC did not recommend revising its recommendation to list ixekizumab for the treatment of patients with nr-AxSpA to increase the maximum quantity (units) from one to two. The PBAC's decision to not recommend was based on, among other matters, the consistent and fair application of the PBAC Guidelines that drugs listed on the General Schedule are to have a maximum quantity per prescription that provides supply for one months' treatment. Sponsor's Comment: The sponsor had no comment.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
LUMACAFTOR WITH IVACAFTOR Sachet containing granules, lumacaftor 150 mg with ivacaftor 188 mg Sachet containing granules, lumacaftor 75 mg with ivacaftor 94 mg Sachet containing granules, lumacaftor 100 mg with ivacaftor 125 mg Orkambi® Vertex Pharmaceuticals (Australia) Pty. Ltd. Category 2 submission (Change to existing listing)	Cystic fibrosis (CF)	To request a Section 100 (Highly Specialised Drugs Program) Authority Required (Written) listing for the treatment of cystic fibrosis patients homozygous for the F508del mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene from aged 1 to less than 2 years.	Recommended	The PBAC recommended that the restriction for lumacaftor with ivacaftor granules be extended to include patients with CF, homozygous for the F580del mutation in the CFTR gene, to include patients aged 1 to less than 2 years. The PBAC also recommended that an additional strength of granules (lumacaftor 75 mg/ivacaftor 94 mg) should be available under Section 100 (Highly Specialised Drugs Program) for use in this population. The PBAC considered that the supporting evidence was limited but acknowledged the difficulties in obtaining efficacy data from paediatric patients. Overall, the PBAC considered that the claim of superior efficacy over best supportive care in patients aged from 1 to less than 2 years was biologically plausible and likely to be beneficial. The PBAC considered lumacaftor with ivacaftor was likely to be cost-effective at a unit price no higher than that of the current PBS listing (for patients ≥ 2 years of age). The PBAC advised that the current risk sharing arrangement financial caps for lumacaftor with ivacaftor should be increased to accommodate patients commencing treatment earlier.
MAVACAMTEN Capsule 2.5 mg Capsule 5 mg Capsule 10 mg Capsule 15 mg Camzyos® Bristol-Myers Squibb Australia Pty Ltd Standard re-entry submission (New listing)	Hypertrophic cardiomyopathy (HCM)	Resubmission to request a General Schedule Authority Required (STREAMLINED) listing for the treatment of adults with symptomatic obstructive HCM.	Not Recommended	The PBAC did not recommend the listing of mavacamten for the treatment of adults with symptomatic obstructive HCM. The PBAC considered that mavacamten ± beta blocker/calcium channel blocker (BB/CCB) provided a moderate and important clinical benefit over standard of care (SOC) BB/CCB alone in terms of symptomatic improvement, however considered a number of issues remained unresolved in the resubmission. These issues included the proposed place in therapy, the uncertain impact of mavacamten on long-term clinical endpoints, such as hospitalisations and mortality, and optimistic assumptions related to clinical benefit in the economic model that were not supported by clinical trial evidence, leading to an uncertain and underestimated incremental cost-effectiveness ratio. The previous submission was considered in November 2022.

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 pathway for this item. Comparator: Standard of care (BB/CCB): The PBAC reaffirmed its previously expressed view that the nominated comparator was appropriate. Clinical claim: Superior effectiveness and inferior safety compared with SOC (BB/CCB): The PBAC considered that mavacamten is likely to provide at least a moderate but important benefit for some patients. The PBAC reiterated that the primary adverse event of concern was reduced left ventricular ejection fraction (LVEF) and that the long-term consequences of treatment compared with SOC were unknown. Economic claim: Cost-utility versus SOC (BB/CCB): The PBAC noted a number of changes were made to the economic model based on the previous consideration, but a number of important issues remained outstanding. Sponsor's Comment: The sponsor welcomes the opportunity to resubmit via the early re-entry pathway and is committed to working with the PBAC to bring mavacamten for the treatment of patients with symptomatic obstructive HCM to Australian patients in a timely manner.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
MIRIKIZUMAB Solution concentrate for I.V. infusion 300 mg in 15 mL Solution for injection 100 mg in 1 mL pre-filled pen Omvoh® Eli Lilly Australia Pty Ltd Category 2 submission (New listing)	Moderate to severe ulcerative colitis (MSUC)	To request General Schedule and Section 100 (Highly Specialised Drugs Program) Authority Required (Written) listings for the treatment MSUC in patients who have had an inadequate response, lost response, or are intolerant/contraindicated to conventional treatments or biologic/targeted synthetic disease-modifying anti-rheumatic drugs (bDMARD/tsDMARD).	Recommended	The PBAC recommended the Section 100 (Highly Specialised Drugs Program) and General Schedule listings of mirikizumab (MIRI) for the treatment of MSUC. The Section 100 recommendation was for listing the vial for intravenous infusion for initial treatment, and the General Schedule listing was for the pre-filled pen for subcutaneous injection for maintenance therapy. The PBAC's recommendation for listing was based on, among other matters, its assessment that the cost-effectiveness of MIRI would be acceptable if it were cost-minimised to the least costly alternative therapy of adalimumab, golimumab, infliximab, ozanimod, tofacitinib, upadacitinib, ustekinumab and vedolizumab. The PBAC noted the submission made a claim that MIRI was of superior comparative effectiveness to adalimumab, however considered based on the evidence presented that a claim of non-inferior comparative effectiveness to all the alternative therapies, including adalimumab, was reasonable. The PBAC noted flow-on restriction changes to the other MSUC bDMARD/tsDMARD listings.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
MOBOCERTINIB Capsule 40 mg Exkivity® Takeda Pharmaceuticals Australia Pty. Ltd. Standard re-entry submission (New listing)	Locally advanced or metastatic non-small cell lung cancer (NSCLC)	Resubmission to request a General Schedule Authority Required (Telephone/Online) listing for the treatment of adults with epidermal growth factor receptor exon 20 insertion (EGFR ex20ins) positive locally advanced or metastatic (Stage IIIB, IIIC or IV) NSCLC who have received platinum-based chemotherapy.	Recommended	The PBAC recommended the General Schedule Authority Required listing of mobocertinib for the treatment of adults with locally advanced (Stage IIIB/IIIC) or metastatic (Stage IV) NSCLC harbouring EGFR ex20ins mutations whose disease has progressed on or after platinum-based chemotherapy. The PBAC was satisfied that mobocertinib provides, for some patients, a significant improvement in efficacy over standard of care. The PBAC considered the amendments made in the resubmission, including changes to the economic model, a reduced price and revised financial estimates had sufficiently addressed the Committee's previous concerns. The PBAC's recommendation for listing was based on, among other matters, its assessment, that the cost-effectiveness of mobocertinib would be acceptable at the price proposed in the submission. The PBAC recommended that flow-on changes to the currently listed gefitinib, erlotinib and afatinib for locally advanced (Stage IIIB) or metastatic (Stage IV) NSCLC to exclude patients with EGFR ex20ins mutations.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
MOLNUPIRAVIR Capsule 200 mg Lagevrio® Merck Sharp & Dohme (Australia) Pty Ltd Matters outstanding (Change to existing listing)	Mild to moderate Coronavirus disease (COVID-19)	To request a General Schedule Authority Required (STREAMLINED) listing for the treatment of high risk patients with mild to moderate COVID-19.	Advice Provided	The PBAC provided advice regarding molnupiravir for the treatment of patients with mild to moderate COVID- 19 who are at high risk of developing severe disease requiring hospitalisation. The PBAC advised that the sponsor's submission did not adequately support the continuation of the current PBS listing and proposed price of molnupiravir, and that additional information is required to inform further consideration of this matter. The PBAC noted the continued toll of the pandemic and the disproportionate effects on older Australians despite vaccination, and the preference expressed by consumers, clinicians and public health experts to maintain molnupiravir on the PBS as an option for patients who could not use nirmatrelvir and ritonavir (Paxlovid®). The PBAC considered all available evidence on the effectiveness and safety of molnupiravir including the randomised trials MOVe OUT and PANORAMIC and noted that the effectiveness in trials as measured by reductions in hospital admissions and mortality ranged between no effect and a modest benefit, PANORAMIC was not considered fully generalisable to the PBS population. The PBAC noted that observational trials showed results consistent with modest benefits, noting that the observational trials were subject to a range of biases. The PBAC considered that the cost-effectiveness of molnupiravir was highly uncertain due to the uncertainty of effectiveness. The PBAC noted that the estimated utilisation and financial impact appeared to be very high relative to the current use (and given PBAC preference that this drug only be used where the more effective treatment (nirmatrelvir and ritonavir) is not suitable). Further clarification was also needed in the restriction regarding the limited patient groups where molnupiravir should be used. The PBAC noted that the current PBS listing would be maintained pending further consideration.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
NIRAPARIB Capsule 100 mg Zejula® GlaxoSmithKline Australia Pty Ltd Matters outstanding (Change to PBS listing)	Epithelial ovarian, fallopian tube, or primary peritoneal cancer	To request an expanded General Schedule Authority Required (Telephone/Online) listing for the treatment of newly diagnosed, homologous recombination deficiency (HRD) positive, breast cancer gene wild type (BRCAwt) advanced (FIGO Stage III-IV) high-grade epithelial ovarian, fallopian tube or primary peritoneal cancer, as deferred at the March 2023 PBAC meeting.	Recommended	The PBAC recommended niraparib for maintenance therapy in patients with newly diagnosed HRD positive BRCAwt advanced epithelial ovarian, fallopian tube or primary peritoneal cancer. The PBAC noted that it had deferred its decision on whether to recommend the proposed listing at the March 2023 PBAC meeting, pending MSAC consideration of HRD testing. Consistent with its March 2023 advice, the PBAC considered that niraparib is likely to be non-inferior to olaparib in the HRD positive BRCAwt setting, as previously accepted in the BRCA mutation setting and therefore could be recommended on the basis of cost-minimisation versus olaparib in the event that olaparib is listed on the PBS for patients with HRD-positive BRCAwt ovarian cancer. The PBAC considered that the outstanding issues were satisfactorily resolved by the revised restriction, revised cost-minimisation analysis and recalculated financial estimates provided by the sponsor. The PBAC noted that the MSAC had recommended HRD testing for determination of eligibility for poly-ADP ribose polymerase inhibitors for this indication at the March 2023 MSAC meeting.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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 Injection concentrate for I.V. infusion 40 mg in 4 mL vial Injection concentrate for I.V. infusion 100 mg in 10 mL vial Opdivo® Bristol-Myers Squibb Australia Pty Ltd Early re-entry submission (Change to existing listing)	Non-small cell lung cancer (NSCLC)	Resubmission to request a Section 100 (Efficient Funding of Chemotherapy) Authority Required (STREAMLINED) listing for the neoadjuvant treatment of resectable NSCLC.	Recommended	The PBAC recommended the listing of nivolumab in combination with chemotherapy for the neoadjuvant treatment of patients with resectable NSCLC. The PBAC considered that the amendments made in the resubmission, including the exclusion of patients with known sensitising epidermal growth factor receptor mutations or anaplastic lymphoma kinase alterations, changes to the economic evaluation and a reduced price had sufficiently addressed the Committee's previous concerns. However, the PBAC considered the utilisation of nivolumab was overestimated and the cost-offsets associated with the current use of immunotherapy in the financial estimates remained underestimated in part due to not accounting for repeat use of immunotherapy in the metastatic setting being precluded. The PBAC considered it would be appropriate to include nivolumab for the proposed population in the current risk sharing arrangement in place for immunotherapies for NSCLC with an increase in the financial caps consistent with the revised financial estimates accepted by the Committee.
NIVOLUMAB Injection concentrate for I.V. infusion 40 mg in 4 mL Injection concentrate for I.V. infusion 100 mg in 10 mL Opdivo® Bristol-Myers Squibb Australia Pty Ltd Department of Health and Aged Care (Commonwealth) (Change to existing listing)	Gastro-oesophageal cancers	To consider an amendment to the current PBS restriction for nivolumab to ensure first-line subsidised access for all patients with advanced or metastatic oesophageal cancers, in addition to human epidermal growth factor receptor-2 negative gastro-oesophageal junction or gastric adenocarcinomas.	Recommended	The PBAC recommended that the current PBS listing for nivolumab be amended to include all patients with squamous cell carcinoma of the oesophagus. The PBAC recalled it had previously noted differences in design and patient populations across the pembrolizumab and nivolumab trials in gastro - oesophageal cancers, but considered that, overall, there was unlikely to be any difference between pembrolizumab and nivolumab in clinical practice for the first line treatment of gastro-oesophageal cancers in terms of clinical benefit, tolerability and treatment duration.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
NUSINERSEN Solution for injection 12 mg in 5 mL Spinraza® Biogen Australia Pty Ltd Standard re-entry submission (Change to existing listing)	Pre-symptomatic spinal muscular atrophy (SMA)	Resubmission to request a Section 100 (Highly Specialised Drug Program) Authority Required (Written) listing for treatment of individuals with pre-symptomatic SMA with 3 copies of the survival motor neuron 2 (*SMN2*) gene, aged less than 18 years.	Recommended	The PBAC recommended the amendment to the current listing of nusinersen to include the pre-symptomatic initiation of nusinersen in patients aged up to less than 36 months, genetically diagnosed with SMA, who have a *SMN2* gene copy number of 3. The PBAC considered that pre-symptomatic initiation of treatment with nusinersen in patients with 3 copies of *SMN2* would provide an additional benefit for some patients compared with initiation upon development of symptoms. The PBAC noted there was remaining uncertainty regarding the cost-effectiveness of pre - symptomatic initiation of nusinersen in patients with 3 copies of *SMN2* due to the uncertain magnitude of incremental benefit compared to symptomatic treatment. However, the PBAC was satisfied that extension of the current listing would be adequately cost-effective with a price reduction for use in the proposed population.
OLAPARIB Tablet 100 mg Tablet 150 mg Lynparza® AstraZeneca Pty Ltd Matters outstanding (Change to PBS listing)	Ovarian cancer	Resubmission to request a General Schedule Authority Required listing for use in combination with bevacizumab for maintenance therapy in patients with newly diagnosed homologous recombination deficiency (HRD) positive breast cancer gene (BRCA) wild type advanced epithelial ovarian, fallopian tube or primary peritoneal cancer, as deferred from the November 2022 PBAC Meeting.	Recommended	The PBAC recommended olaparib for use in combination with bevacizumab for maintenance therapy in patients with newly diagnosed HRD positive BRCA wild type advanced epithelial ovarian, fallopian tube or primary peritoneal cancer. The PBAC noted that it had deferred its decision on whether to recommend the proposed listing at the November 2022 PBAC meeting, pending MSAC consideration of HRD testing. The PBAC noted that the MSAC had recommended HRD testing for determination of eligibility for poly-ADP ribose polymerase inhibitors for this indication at its March 2023 meeting. The PBAC considered that the outstanding issues were satisfactorily resolved by the revised economic evaluation which included a price reduction to account for remaining uncertainty in the modelled benefit of olaparib, as requested by the PBAC in November 2022. The PBAC noted the revised financial estimates appropriately incorporated the reduced olaparib price.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
OLIPUDASE ALFA Powder for I.V. infusion 20 mg Xenpozyme® Sanofi-Aventis Australia Pty Ltd Category 1 submission (New listing)	Acid sphingomyelinase deficiency (ASMD)	To request a Section 100 (Highly Specialised Drugs Program) Authority Required (Written) listing for the treatment of ASMD.	Not Recommended	The PBAC did not recommend the Section 100 (Highly Specialised Drugs Program) listing of olipudase alfa for the treatment of ASMD type A/B or type B. The PBAC considered olipudase alfa was an effective treatment for ASMD type A/B or type B; however, the incremental cost-effectiveness ratio for olipudase alfa compared to best supportive care was extremely high and uncertain. <u>Sponsor's Comment:</u> The sponsor had no comment.
ONASEMNOGENE ABEPARVOVEC Solution for injection, customised based on patient weight Zolgensma® Novartis Pharmaceuticals Australia Pty Limited Standard re-entry submission (Change to existing listing)	Pre-symptomatic spinal muscular atrophy (SMA)	Resubmission to request a Section 100 (Highly Specialised Drug Program) Authority Required (Written) listing for the pre-symptomatic treatment of babies with SMA and 3 copies of the survival motor neuron 2 (*SMN2*) gene.	Recommended	The PBAC recommended the amendment to the current listing of onasemnogene abeparvovec (ONA) to include the pre-symptomatic treatment in patients aged up to 9 months, genetically diagnosed SMA, who have *SMN2* gene copy number of 3. The PBAC considered that pre - symptomatic treatment with ONA in patients with 3 copies of *SMN2* would provide an additional benefit for some patients compared with initiation of treatment upon development of symptoms. The PBAC noted there was remaining uncertainty regarding the cost-effectiveness of pre-symptomatic treatment with ONA in patients with 3 copies of *SMN2* due to the uncertain magnitude of incremental benefit compared to symptomatic treatment. However, the PBAC was satisfied that extension of the current listing would be adequately cost-effective with a price reduction for use in the proposed population.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
PATISIRAN Solution concentrate for I.V. infusion 10 mg in 5 mL Onpattro® Alnylam Australia Pty Ltd Category 1 submission (New listing)	Hereditary transthyretin-mediated amyloidosis (hATTR amyloidosis)	To request a Section 100 (Highly Specialised Drugs Program) Authority Required (Written) listing for the treatment of hATTR amyloidosis in adult patients with stage 1 or stage 2 polyneuropathy.	Not Recommended	The PBAC did not recommend patisiran for treatment of patients with hATTR amyloidosis with polyneuropathy. The PBAC considered there was a high unmet need in the requested patient population, and that efficacy and safety of patisiran compared with best supportive care had been demonstrated by the clinical evidence, although there was a limited duration of randomised evidence. The PBAC considered that the submission had not demonstrated patisiran is cost - effective. The PBAC nominated the early resolution re-submission pathway for this item. <u>Sponsor's Comment:</u> The sponsor had no comment.
PEMBROLIZUMAB Solution concentrate for I.V. infusion 100 mg in 4 mL Keytruda® Merck Sharp & Dohme (Australia) Pty Ltd Early re-entry submission (Change to existing listing)	Breast cancer	Resubmission to request a Section 100 (Efficient Funding of Chemotherapy) Authority Required (STREAMLINED) listing for the treatment of early stage triple negative breast cancer (eTNBC) in patients who have not received prior systemic therapy.	Recommended	The PBAC recommended the listing of pembrolizumab for the treatment of eTNBC. The PBAC noted there is a high clinical need for effective treatment in this patient population, who are typically young and have a poor prognosis. The PBAC considered that the amendments made in the resubmission, including changes to the economic evaluation and a reduced price had sufficiently addressed the Committee's previous concerns. The PBAC considered the financial estimates should be revised to be based on the same number of pembrolizumab treatment cycles as for the economic model, and to account for offsets due to reduced use of pembrolizumab in the metastatic setting. The PBAC considered a risk sharing arrangement including use in both the early stage and metastatic settings to be appropriate.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
POMALIDOMIDE Capsule 1 mg Capsule 2 mg Pomolide™ Juno Pharmaceuticals Pty Ltd Category 4 submission (New listing)	Relapsed/refractory multiple myeloma (RRMM)	To request a Section 100 (Highly Specialised Drugs Program) Authority Required listing for the treatment of RRMM of two new forms under the same conditions as the currently listed forms of pomalidomide.	Recommended	The PBAC recommended a Section 100 (Highly Specialised Drugs Program) Authority Required listing of two new forms of pomalidomide (capsule 1 mg, capsule 2 mg; Pomolide™) for the treatment of RRMM under the same conditions as the currently listed forms of pomalidomide (capsule 3 mg, capsule 4 mg; Pomolide™).

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
RAVULIZUMAB Solution concentrate for I.V. infusion 300 mg in 3 mL vial Solution concentrate for I.V. infusion 1.1 g in 11 mL vial Ultomiris® Alexion Pharmaceuticals Australasia Pty Ltd Matters outstanding (Change to PBS listing)	Atypical haemolytic uraemic syndrome (aHUS)	To request a Section 100 (Highly Specialised Drugs Program) Authority Required (Written) listing for the treatment of aHUS, as deferred from the March 2023 PBAC Meeting.	Recommended	The PBAC recommended the Authority required listing of ravulizumab for the treatment of aHUS, on the basis that it should be available only under special arrangements under the Section 100 Highly Specialised Drugs Program, following its deferral at the March 2023 PBAC meeting. The PBAC had previously noted that funding of PBS medicines for public hospital inpatients was inconsistent with PBS policy and considered that ravulizumab should not be subsidised for admitted patients in public hospitals. Following the March 2023 meeting, the PBAC sought advice from the aHUS Expert Reference Group to resolve issues for the aHUS PBS restrictions for C5 inhibitor therapies (ravulizumab and eculizumab) to allow appropriate PBS use of ravulizumab. This advice informed the recommended PBS listing for ravulizumab in July 2023. The PBAC also considered its additional prior concerns related to the cost-minimisation approach and the financial estimates were adequately resolved. The PBAC considered ravulizumab would be cost-effective on the basis of cost-minimisation to eculizumab. The PBAC also considered that the overall PBS cost for aHUS treatment with a C5 inhibitor therapy should not increase with the listing of ravulizumab. The PBAC recommended that a risk sharing arrangement to cover the overall PBS cost of aHUS treatment with a C5 inhibitor therapy would be required.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
RAVULIZUMAB Solution concentrate for I.V. infusion 300 mg in 3 mL Solution concentrate for I.V. infusion 1,100 mg in 11 mL Ultomiris® Alexion Pharmaceuticals Australasia Pty Ltd Category 2 submission (Change to existing listing)	Paroxysmal nocturnal haemoglobinuria (PNH)	To request a Section 100 (Highly Specialised Drugs Program) Authority Required (Written) listing for paediatric patients with PNH.	Recommended	The PBAC recommended amending the Section 100 Highly Specialised Drugs Program (Public and Private Hospital) listings of ravulizumab to permit use in paediatric patients with PNH. In making this recommendation, the PBAC was satisfied that the available evidence, while limited due to the rarity of PNH in children, was overall reasonable to conclude that ravulizumab is likely to be of non-inferior comparative effectiveness and safety to eculizumab in this patient population. The PBAC noted the extension to the PBS listing to include paediatric patients would resolve current equity issues for these patients by providing an effective treatment option with reduced hospital attendances and infusion frequency, which can be highly impactful on children.
RELATLIMAB AND NIVOLUMAB Solution concentrate for I.V. infusion containing 80 mg relatlimab and 240 mg nivolumab in 20 mL vial Opdualag® Bristol-Myers Squibb Australia Pty Ltd Matters arising (New PBS listing)	Melanoma	Resubmission to request a Section 100 (Efficient Funding of Chemotherapy) Authority Required (STREAMLINED) listing for the treatment of unresectable Stage III or Stage IV malignant melanoma.	Recommended	The PBAC recommended the listing of relatlimab and nivolumab under the Section 100 Highly Specialised Drugs (HSD) Program, while implementation issues relating to a Section 100 Efficient Funding of Chemotherapy (EFC) listing were being worked through. The PBAC noted that under S100 EFC, patients are not required to pay a co-payment for repeat prescriptions, but would be required to do so under S100 HSD. The PBAC considered that this should not be a barrier to a S100 HSD listing, noting that absence of PBS access was a greater equity issue than a S100 HSD vs S100 EFC listing.

DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION	DRUG TYPE AND USE	LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION	PBAC OUTCOME	
RIMEGEPANT Tablet 75 mg Nurtec ODT® Pfizer Australia Pty Ltd Category 2 submission (New listing)	Acute migraine attacks	To request a General Schedule Authority Required (STREAMLINED) listing for adults with migraine who have not responded adequately to treatment of at least two triptans.	Not Recommended	The PBAC did not recommend rimegepant for the acute treatment of adults with migraine who have not responded adequately, are intolerant or contraindicated to analgesics and at least two selective 5-hydroxytryptamine receptor agonists (triptans). The PBAC acknowledged that there was a clinical need for new, oral treatments for the acute treatment of migraine and considered that rimegepant provided a benefit over best supportive care in terms of efficacy. However, the PBAC considered that rimegepant was not cost-effective at the price proposed in the submission noting that the economic model included optimistic assumptions and that a number of the modelled inputs required revision. The PBAC considered that a risk sharing arrangement would be required to manage the uncertainty with the financial estimates. The PBAC nominated the early re-entry submission pathway for this item. <u>Sponsor's Comment:</u> While disappointed with the PBAC's decision not to recommend rimegepant for the treatment of acute migraine attacks, Pfizer welcomes the PBAC's acknowledgment of the burden of acute migraine and clinical unmet need for novel effective treatments such as rimegepant. Pfizer looks forward to continuing to work with the PBAC and the Department of Health and Aged Care to provide access to rimegepant for patients suffering from acute migraine attacks.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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 Powder for injection 180 mg Trodelvy® Gilead Sciences Pty Limited Category 2 submission (Change to existing listing)	Hormone receptor-positive (HR+) human epidermal growth factor receptor-2 negative (HER2-) advanced or metastatic breast cancer	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 HR+, HER2- breast cancer, who have previously received at least two systemic therapies, one of which may have been in the neoadjuvant/adjuvant setting.	Not Recommended	The PBAC did not recommend the listing of sacituzumab govitecan, for the treatment of patients with unresectable locally advanced or metastatic HR+, HER2- breast cancer, who have previously received at least two systemic therapies. The PBAC considered that sacituzumab govitecan provided a modest clinical benefit, with the magnitude likely depending on the population treated. The PBAC considered that the proposed restriction was not consistent with the pivotal TROPiCS-02 trial, the proposed TGA listing, or international treatment guidelines. The PBAC advised that the proposed PBS population should reflect the patients included in TROPiCS-02 which was a heavily pre-treated patient population. The PBAC considered sacituzumab govitecan was not cost-effective at the price proposed in the submission given optimistic assumptions included in the economic model. The PBAC nominated the early re-entry pathway for this item. <u>Sponsor's Comment:</u> The sponsor had no comment.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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 Solution for injection 300 mg in 2 mL pre filled syringe Solution for injection 150 mg in 1 mL pre filled syringe Solution for injection 150 mg in 1 mL pre filled pen Solution for injection 300 mg in 2 mL pre filled pen Cosentyx® Novartis Pharmaceuticals Australia Pty Limited Category 2 submission (Change to existing listing)	Hidradenitis Suppurativa (HS)	To request a General Schedule Authority Required (Written) listing for the treatment of HS.	Not Recommended	The PBAC did not recommend the General Schedule, Authority Required listing of secukinumab for the treatment of moderate to severe HS. The Committee considered there was a high clinical need for additional therapies for the treatment of HS. However, the PBAC considered the available evidence suggests that while secukinumab provides a modest benefit compared to placebo, the clinical claim in the submission that secukinumab was non - inferior to adalimumab in terms of effectiveness was uncertain. The PBAC considered the extent of uncertainty regarding the effectiveness claim may be acceptable in the context of the high clinical need if there was additional certainty regarding the cost minimised price for secukinumab and the net PBS/RPBS cost. The PBAC nominated the early re-entry pathway for secukinumab. <u>Sponsor's Comment:</u> Novartis is pleased to see the PBAC recognised there was a high clinical need for additional therapies for the treatment of HS and will continue to work with the PBAC to enable earliest possible access to secukinumab for HS patients.

DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION	DRUG TYPE AND USE	LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION	PBAC OUTCOME	
SELPERCATINIB Capsule 40 mg Capsule 80 mg Retevmo® Eli Lilly Australia Pty Ltd Category 1 submission (New listing)	Non-small cell lung cancer (NSCLC)	To request a General Schedule Authority Required (STREAMLINED) listing for the treatment of rearranged during transfection (*RET*) fusion-positive, advanced or metastatic NSCLC, irrespective of line of therapy.	Not Recommended	The PBAC did not recommend the listing of selpercatinib for the treatment of *RET* fusion-positive, locally advanced or metastatic NSCLC, irrespective of line of therapy. The PBAC acknowledged the clinical need for effective treatments for patients with this condition. The Committee considered the results of the single arm LIBRETTO-001 trial demonstrated clinical activity for selpercatinib. However, the PBAC advised that indirect comparisons with pembrolizumab in combination with pemetrexed + platinum-based chemotherapy were associated with a high degree of uncertainty and were unable to support a claim of superior comparative effectiveness. Given the limitations of the comparative clinical data, the PBAC considered the estimated incremental cost-effectiveness ratio was highly uncertain. <u>Sponsor's Comment:</u> Eli Lilly wishes to thank all of the healthcare professionals, professional societies, patient organisations and consumers for their support of the selpercatinib (Retevmo®) submission. Selpercatinib has received provisional approval from the TGA for the treatment of patients with *RET* fusion-positive NSCLC. While Eli Lilly welcomes the PBAC's acknowledgement of the clinical unmet need for these patients and clinical activity of selpercatinib, we are disappointed by the PBAC's decision not to recommend the PBS listing of selpercatinib for the treatment of patients with *RET* fusion-positive NSCLC. Eli Lilly remains committed to making selpercatinib accessible for patients with *RET* fusion-positive NSCLC.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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 Injection 5 mg in 1.5 mL pre-filled pen Injection 10 mg in 1.5 mL pre-filled pen Injection 15 mg in 1.5 mL pre-filled pen Sogroya® Novo Nordisk Pharmaceuticals Pty. Limited Category 2 submission (New listing)	Paediatric growth hormone deficiency (GHD)	To request a Section 100 (Growth Hormone Program) Authority Required (Written) listing for the treatment of paediatric patients with GHD.	Recommended	The PBAC recommended the Section 100 Growth Hormone Program listing of somapacitan on a cost - minimisation basis to somatrogon and somatropin for paediatric patients with GHD, with the equi-effective doses being: • 1 mg of somatrogon is equivalent to 0.240 mg of somapacitan • 1 mg of somatropin is equivalent to 0.665 mg of somapacitan.
SONIDEGIB Capsule 200 mg Odomzo® Sun Pharma ANZ Pty Ltd Category 3 submission (Other matters)	Metastatic or locally advanced basal cell carcinoma (BCC)	To request the PBAC consider the previously estimated utilisation for sonidegib and vismodegib for the treatment of metastatic or locally advanced BCC.	Advice Provided	The PBAC provided advice on the risk sharing arrangement for sonidegib (Odomzo®) and vismodegib (Erivedge®) for the treatment of metastatic or locally advanced BCC.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
TAFAMIDIS Capsule 61 mg Vyndamax® Pfizer Australia Pty Ltd Standard re-entry submission (New listing)	Transthyretin amyloid cardiomyopathy	Resubmission to request a General Schedule Authority Required (Written) listing for the treatment of transthyretin amyloid cardiomyopathy.	Recommended	The PBAC recommended the listing of tafamidis for patients with transthyretin amyloid cardiomyopathy, with New York Heart Association (NYHA) class I-II. The PBAC considered that there was a clear clinical benefit for tafamidis, including a reduction in mortality. The PBAC considered that the incremental cost-effectiveness ratio was high, but would be acceptable at the lower price proposed in the pre-PBAC response, in the context of the high unmet need for this patient population. The PBAC considered that a risk sharing arrangement would be required due to a high risk of utilisation outside the proposed restriction and to address uncertainty around the overall financial impact.
TAGRAXOFUSP Solution concentrate for I.V. infusion 1 mg in 1 mL Elzonris® A.Menarini Australia Pty Limited Category 1 submission (New listing)	Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN)	To request a Section 100 (Efficient Funding of Chemotherapy Program) Authority Required (Telephone/Online) listing for the treatment of BPDCN.	Not Recommended	The PBAC did not recommend tagraxofusp for the first - line treatment of BPDCN. The PBAC noted that BPDCN was a very rare disease with a poor prognosis, but considered that, based on the data presented, tagraxofusp did not demonstrate superiority over standard chemotherapy. The PBAC considered that the revised incremental cost-effectiveness ratio presented in the pre-PBAC response was very high and highly uncertain. The PBAC considered that the estimated utilisation and financial impact estimates were uncertain. <u>Sponsor's Comment:</u> Menarini is disappointed with the PBAC outcome and commits to working with the PBAC and the Department of Health and Aged Care to make Elzonris® available for patients with BPDCN which is a very rare, hard to treat and aggressive malignancy with poor prognosis.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
TIRZEPATIDE Injection 2.5 mg in 0.5 mL Injection 5 mg in 0.5 mL Injection 7.5 mg in 0.5 mL Injection 10 mg in 0.5 mL Injection 12.5 mg in 0.5 mL Injection 15 mg per 0.5 mL Mounjaro® Eli Lilly Australia Pty Ltd Category 2 submission (New listing)	Type 2 Diabetes Mellitus (T2D)	To request a General Schedule Authority Required (Telephone/Online) listing for the treatment of adult patients with inadequately controlled T2D as dual therapy in combination with metformin.	Not Recommended	The PBAC did not recommend tirzepatide for the treatment of adult patients with inadequately controlled T2D as dual therapy in combination with metformin. The PBAC considered that tirzepatide 10 mg once weekly and tirzepatide 15 mg once weekly was superior in terms of effectiveness for glycaemic benefits and short-term weight loss compared to semaglutide 1 mg once weekly, but this claim was not supported for tirzepatide 5 mg once weekly compared to semaglutide 1 mg once weekly. The PBAC considered the non - inferior safety claim was not adequately supported for any of the comparisons. The PBAC considered that the incremental cost-effectiveness ratio was high, inadequately justified, and uncertain. The PBAC advised that a revised economic model including a substantial price reduction would be required for the proposed listing to be considered cost-effective. The PBAC also considered that the financial impact was extremely high at the requested price and uncertain. <u>Sponsor's Comment:</u> Eli Lilly wishes to thank all of the healthcare professionals, professional societies, leadership bodies, patient organisations and consumers for their support of the tirzepatide (Mounjaro®) submission. We are disappointed by the PBAC's decision not to recommend the PBS listing of tirzepatide for the treatment of adult patients with inadequately controlled T2D as dual therapy in combination with metformin. Eli Lilly remains committed to making this medicine accessible for adult patients living with T2D.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
TRASTUZUMAB DERUXTECAN Powder for I.V. infusion 100 mg Enhertu® AstraZeneca Pty Ltd Matters outstanding (New PBS listing)	Breast Cancer	To consider the population deferred from the March 2023 PBAC consideration for the resubmission to request a Section 100 (Efficient Funding of Chemotherapy Program) Authority Required (Written) listing for the treatment of human epidermal growth factor receptor 2 positive (HER2) metastatic breast cancer in patients whose disease has progressed following treatment with at least one prior HER2-directed regimen in the metastatic setting or whose disease has progressed during or within 6 months following HER2-directed adjuvant treatment.	Recommended	The PBAC recommended the listing of trastuzumab deruxtecan (T-DXd) for the treatment of HER2 positive breast cancer for patients who have progressed following treatment with at least one prior HER2 directed regimen for metastatic disease, or relapsed during or within 6 months of receiving HER2 directed adjuvant therapy. The recommendation pertained to the deferred population from the March 2023 consideration (those who would receive T-DXd in a third and later line setting), which is additional to the recommended population at the March meeting, to allow for a single restriction that includes all patients for whom the PBAC identified as clinically appropriate for treatment with T-DXd. The PBAC was satisfied that revisions to the financial estimates reflected that later line patients would be less likely to undergo treatment with T-DXd at the same rate as earlier line patients due to fitness-for-treatment and toxicity concerns. The PBAC noted that the potential for use in a broader patient population where the use may not be cost - effective should be managed through a risk sharing arrangement.
TRIENTINE Capsule containing trientine dihydrochloride 250 mg (equivalent to 166.7 mg trientine) Trientine Dr.Reddy's® Dr Reddy's Laboratories (Australia) Pty Ltd Category 3 submission (New listing)	Wilson disease	To request a General Schedule Authority Required (Telephone/Online) listing for the treatment of Wilson disease under the same conditions as the currently listed brand.	Recommended	The PBAC recommended the listing of Trientine Dr.Reddy's® brand of trientine dihydrochloride 250 mg capsules under the same circumstances as the PBS - listed brand of trientine dihydrochloride 250 mg capsules, Trientine Waymade®. The PBAC considered that these products should be marked as equivalent in the Schedule of Pharmaceutical Benefits for the purposes of substitution.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
UPADACITINIB Tablet 15 mg Tablet 30 mg Tablet 45 mg Rinvoq® Abbvie Pty Ltd Category 2 submission (Change to PBS listing)	Crohn disease (CD)	To request a General Schedule Authority Required (Written) listing for the treatment of severe CD.	Recommended	The PBAC recommended the General Schedule listing of upadacitinib for the treatment of CD. The PBAC's recommendation was based on, among other matters, its assessment the cost-effectiveness of upadacitinib would be acceptable if it were cost - minimised to the least costly alternative therapy of adalimumab, infliximab, vedolizumab and ustekinumab. The PBAC noted flow-on changes to other CD listings to include upadacitinib in the list of eligible therapies.

DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION	DRUG TYPE AND USE	LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION	PBAC OUTCOME	
USTEKINUMAB Solution concentrate for I.V. infusion 130 mg in 26 mL Solution for injection 90 mg in 1 mL pre-filled syringe Stelara® Janssen-Cilag Pty Ltd Category 2 submission (Change to existing listing)	Fistulising Crohn disease (CD)	To request a Section 100 (Highly Specialised Drugs Program) Authority Required (Written) IV infusion listing, and a General Schedule Authority Required (Written) subcutaneous injection listing, for the treatment of patients with complex refractory fistulising CD.	Recommended	The PBAC recommended the Section 100 (Highly Specialised Drugs Program) and General Schedule listings of ustekinumab (UST) for the treatment of fistulising CD. The PBAC's recommendation for listing was based on, among other matters, its assessment that the cost-effectiveness of UST would be acceptable if it were cost-minimised to the least costly alternative therapy of infliximab (IFX) and adalimumab (ADA). The PBAC considered there was a high clinical need for additional effective therapies for the treatment of fistulising CD for patients who do not otherwise meet the criteria for PBS-subsidised treatment for severe CD, where more biologic or targeted synthetic disease modifying anti-rheumatic drugs (bDMARDs/tsDMARDs) are available. The PBAC also noted the comments received from health professionals and organisations outlined the severe impacts of fistulising CD on quality of life and the risks of repeat surgeries, which can often end in proctectomy and the need for a permanent stoma, further highlighted this need for additional therapeutic options. The PBAC considered that while the evidence supporting the clinical claims of non-inferior efficacy and safety versus the nominated comparators was limited, on balance when considering the clinical need for additional therapies for fistulising CD, the claims versus IFX and ADA were likely to be reasonable. The PBAC noted flow-on changes to other fistulising CD listings to include UST in the list of eligible therapies.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 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	
VARICELLA ZOSTER VIRUS RECOMBINANT VACCINE Injection [1 vial] & adjuvant substance diluent [0.5 mL vial] Shingrix® GlaxoSmithKline Australia Pty Ltd Early resolution submission (New listing)	Prevention of herpes zoster (HZ) and post-herpetic neuralgia (PHN)	Resubmission to request a National Immunisation Program (NIP) listing for the prevention of herpes zoster and post - herpetic neuralgia, for non - Indigenous individuals aged 65 to 69 years and individuals aged 71 years and older.	Recommended	The PBAC recommended that varicella zoster virus recombinant vaccine (RZV, Shingrix®) be a designated vaccine for the purposes of the *National Health Act 1953*, for the prevention of HZ and for individuals aged 65 years (primary program) and older (catch-up program). The PBAC considered, based on the reduced price proposed, that RZV is cost-effective with the age for the primary program lowered to 65 years (compared with the March 2023 recommendation for vaccination at age 70 years) and with there being no upper age limit for the catch-up program. The PBAC noted the cost - effectiveness relies on accepting the long term modelled vaccine efficacy, and in this context considered that the cost-effectiveness of RZV should be reassessed if a booster dose is required or if long-term efficacy is less than predicted. The PBAC noted the total cost of the program was high. RZV will be listed on the NIP as of the 1 November 2023 for non - Indigenous individuals aged 70 years, Aboriginal and Torres Strait Islander individuals aged ≥ 50 years and immunocompromised individuals aged ≥ 18 years with conditions at high risk of HZ infection (based on the PBAC's March 2023 recommendation).
ZOLEDRONIC ACID Injection concentrate for I.V. infusion 4 mg (as monohydrate) in 5 mL APO-Zoledronic Acid® Apotex Pty Ltd Department of Health and Aged Care (Commonwealth) (Change to listing)	Adjuvant treatment of breast cancer	To request that the PBAC advise whether the circumstances under which zoledronic acid is listed on the PBS be expanded to include the adjuvant treatment of breast cancer in post - menopausal women	Recommended	The PBAC recommended amending the circumstances under which zoledronic acid is available on the PBS to include its use for the adjuvant management of breast cancer in post - menopausal women. The PBAC recommended this listing on the basis of its assessment that the cost-effectiveness of zoledronic acid would be acceptable in this population at an equivalent price to the existing listing.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 PBAC MEETING

DRUG NAME, FORM(S), STRENGTH(S) AND SPONSOR	DRUG TYPE AND USE	LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION	PBAC OUTCOME
ADALIMUMAB Injection 20 mg in 0.4 mL pre-filled syringe Injection 40 mg in 0.8 mL pre-filled syringe Injection 40 mg in 0.8 mL pre-filled pen Abrilada® Pfizer Australia Pty Ltd	Crohn disease, Ulcerative colitis, Juvenile idiopathic arthritis, Rheumatoid arthritis, Psoriatic arthritis, Ankylosing spondylitis, Plaque psoriasis, Hidradenitis suppurativa	Review of positive PBAC recommendations not accepted by applicants	The PBAC advised that the recommendation be extended for an additional 12 months.
CERTOLIZUMAB PEGOL Injection 200 mg in 1 mL pre-filled syringe pen Solution for injection 200 mg in 1 mL pre-filled pen Cimzia® UCB Australia Pty Ltd	Severe chronic plaque psoriasis	Review of positive PBAC recommendations not accepted by applicants	The PBAC advised that the March 2019 recommendation could be revoked.
ENOXAPARIN Injection containing enoxaparin sodium 120 mg (12,000 I.U. anti-Xa) in 0.8 mL pre-filled syringe Injection containing enoxaparin sodium 150 mg (15,000 I.U. anti-Xa) in 1 mL pre-filled syringe Clexane Forte® Enoxaparin Winthrop® Clexane Forte Safety Lock® Sanofi-Aventis Australia Pty Ltd	Antithrombotic agent	Review of positive PBAC recommendations not accepted by applicants	The PBAC advised that the recommendation be extended for an additional 12 months.
GUSELKUMAB Solution for injection 100 mg in 1 mL pen device Tremfya® Janssen-Cilag Pty Ltd	Chronic plaque psoriasis	Review of positive PBAC recommendations not accepted by applicants	The PBAC advised that the July 2020 recommendation could be revoked, noting that there was an alternate presentation of the product listed on the PBS.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 PBAC MEETING

DRUG NAME, FORM(S), STRENGTH(S) AND SPONSOR	DRUG TYPE AND USE	LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION	PBAC OUTCOME
INSULIN ASPART Injections (human analogue), cartridges, 100 units per mL, 3 mL Fiasp® Novo Nordisk Pharmaceuticals Pty Ltd	Diabetes mellitus	Review of positive PBAC recommendations not accepted by applicants	The PBAC advised that the recommendation be extended for an additional 12 months.
TRABECTEBIN Powder for I.V. infusion 0.25 mg Yondelis® Specialised Therapeutics Pharma Pty Ltd	Leiomyosarcoma	Review of positive PBAC recommendations not accepted by applicants	This item was withdrawn. Listing arrangements are currently under consideration.

DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION	DRUG TYPE AND USE	LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION	PBAC OUTCOME
CANCER MEDICINES Multiple forms and strengths Multiple sponsors	Cancer	To provide the PBAC with a report on the use of surrogate outcome measures in PBAC submissions for cancer medicines	Surrogate outcome measures have an increasingly important role in cancer medicine research providing evidence to support registration and subsidy of new medicines. The PBAC recommended a research project into the use of surrogate outcome measures in PBAC submissions for cancer medicines in May 2022. The PBAC noted the draft report, 'A review of cancer related surrogate outcomes used for PBAC decision making' (the Report), prepared by Monash University under contract to the Department. The Report showed that 50% (247 out of 498) of PBAC submissions (including resubmissions) for cancer medicines between January 2012 and May 2022, were based primarily on a surrogate outcome. Forty-four per cent of these were not recommended for PBS-listing by the PBAC, and in 62% (67 out of 108) of cases this was due to immature overall survival (OS) data. In submissions that presented modelled economic evaluations, surrogate outcomes were used in 85% (140 out of 165) of cases. However, a literature review of surrogate outcome measure validation studies presented mixed results with most studies showing low to moderate correlation between OS and the most common surrogate measures, including progression-free survival and objective/overall response rate. The PBAC considered that the Report was informative for health technology assessment and recommended that the Report and PBAC consideration should be published on the PBS website and be provided to relevant stakeholders.

DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION	DRUG TYPE AND USE	LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION	PBAC OUTCOME
PBS Restrictions for Type 2 diabetes mellitus medicines PIOGLITAZONE DULAGLUTIDE SEMAGLUTIDE EMPAGLIFLOZIN DAPAGLIFLOZIN ALOGLIPTIN SAXAGLIPTIN SITAGLIPTIN LINAGLIPTIN VILDAGLIPTIN All forms and strengths	Type 2 diabetes mellitus (T2DM) medicines	To review the PBS restrictions for T2DM medicines following stakeholder consultation on the restriction changes recommended by the PBAC in March 2023.	The PBAC noted the comments received from a range of T2DM stakeholders on the proposed changes to the PBS restrictions for T2DM medicines. The PBAC noted that stakeholders generally supported the simplification of the restrictions, and clarification of combinations of medicines that are not PBS-subsidised. The PBAC recommended that the level of restriction for pioglitazone be changed to a Restricted Benefit listing, without any additional clinical criteria. The PBAC recommended removal of the requirement for contraindication or intolerance to metformin for patients to use dipeptidyl peptidase 4 (DPP4) inhibitors, sodium-glucose cotransporter 2 (SGLT2) inhibitors and glucagon-like peptide 1 receptor agonists (GLP-1 RAs) in dual therapy with insulin. The PBAC recommended alignment of the PBS restrictions for DPP4 inhibitors, no longer restricting the use of some of these medicines in combination with insulin or SGLT2 inhibitors. The PBAC recommended that the Authority Type for GLP-1 RAs, for therapy initiation for all indications, be changed from Authority Required (STREAMLINED) to Authority Required (Telephone/Online), but that continuing access should be via a streamlined authority. The PBAC further recommended that the use of GLP-1 RAs in all T2DM indications should be restricted to patients who are contraindicated or intolerant to an SGLT2 inhibitor, or who do not achieve a clinically meaningful glycaemic response with an SGLT2 inhibitor. The PBAC considered that it may be appropriate to expand the PBS listings for GLP-1 RAs to include use in combination with metformin, a sulfonylurea or insulin, for T2DM patients with a body mass index (BMI) greater than 35 kg/m^2, without a requirement to trial an SGLT2 inhibitor. The PBAC requested that the Department provide financial estimates for this expanded listing to be considered at a future meeting.
PBS Restrictions for Growth Hormones SOMATROPIN Genotropin GoQuick® Pfizer Australia Pty Ltd Norditropin FlexPro® Novo Nordisk Pharmaceuticals Pty Limited NutropinAq® Ipsen Pty Ltd	Growth hormone medicines	Request to remove references to Prader Willi Syndrome (PWS) present in the PBS restrictions in the *National Health (Growth Hormone Program) Special Arrangement 2015* to align with the updated definitions of 'adult' and 'child'.	The PBAC recommended removing references to PWS present in the PBS restrictions for somatropin (PBS item codes: 11650E, 11495B, 11493X, 11895C) in the *National Health (Growth Hormone Program) Special Arrangement 2015* to align with the updated definitions of 'adult' and 'child'. The updated definitions of 'adult' and 'child' are being amended to align with the intent of previous PBAC recommendations from July and August 2019. The PBAC advised that this would have no impact on patients currently receiving PBS subsidised growth hormone treatment.

Version 2
Amendment
1. FOSLEVODOPA WITH FOSCARBIDOPA - Web outcome amended.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
JULY 2023 PBAC MEETING

Submission category types

Category 1	A request for PBS or NIP listing of one or more of the following: • A first in class medicine or vaccine, and/or a medicine or vaccine for a new population. OR • A drug with a codependent technology that requires an integrated codependent submission to the PBAC and MSAC. OR • 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: • Listing of a new pharmaceutical item of a listed medicine. • Consideration as an exempt item (Exempt item as per subsection 84AH of the *National Health Act 1953*). • Including a listed medicine on the prescriber bag, or varying an existing prescriber bag listing. • A change/new manner of administration of a listed medicine. • A change to the maximum quantity and/or number of repeats of a listed medicine. • 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: • New or varied listed drugs, medicinal preparations and designated vaccines that pose no greater risk • Pharmaceutical benefits that can no longer be supplied early • 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: • an applicant chooses not to accept the PBAC nominated resubmission pathway; or • an Early Re-entry or Early Resolution Pathway has been nominated by the PBAC and an applicant decides to address issues other than those identified by the PBAC (including a subset of issues); or • an applicant decides to lodge later than the allowable timelines for the other pathways.
Early re-entry pathway	An Early Re-entry Pathway may be nominated by the PBAC where the PBAC considers that the remaining issues could be easily resolved and the medicine or vaccine does not represent High Added Therapeutic Value (HATV) for the proposed population. Applicants who accept this pathway are eligible for PBAC consideration at the immediate next meeting.
Early resolution pathway	For medicines or vaccines deemed by the PBAC to represent HATV AND where the PBAC considers that the remaining issues could be easily resolved, including when: • new clinical study data requiring evaluation is not considered necessary by the PBAC to support new clinical claims to be made in the resubmission; and • a revised model structure or input variable changes (beyond those specified by the PBAC) are not necessary to support any new economic claims, or to estimate the utilisation and financial impacts to be made in the resubmission. Applicants who accept this pathway are eligible for PBAC consideration out-of-session (before the main meeting), unless the department, in consultation with the PBAC Chair, identifies an unexpected issue such that the resubmission needs consideration at the next main PBAC meeting.
Facilitated resolution pathway	A Facilitated Resolution Pathway may be nominated by the PBAC where the PBAC considers the issues for resolution could be explored through a workshop AND where the medicine or vaccine meets the HATV criteria. Applicants who accept this pathway are eligible for a solution-focussed workshop with one or more members of the PBAC. The workshop agenda will be based on the issues for resolution outlined in the PBAC Minutes. This can be further clarified during the post-PBAC meeting with the Chair.