PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
ABIRATERONE AND METHYLPREDNISOLONE Pack containing 120 tablets abiraterone (as acetate) 125 mg and 60 tablets methylprednisolone 4 mg Yonsa® Mpred™ SUN PHARMA ANZ PTY LTD Standard re-entry submission (New PBS listing)	Prostate cancer	Resubmission to request a General Schedule Authority Required listing of a composite pack for the treatment of castration-resistant metastatic carcinoma of the prostate.	Recommended	The PBAC recommended the General Schedule Authority Required listing of the composite pack comprising abiraterone acetate tablets in a fine particle formulation and oral methylprednisolone tablets (Yonsa® MPRED™) for the treatment of patients with metastatic castration resistant prostate cancer (mCRPC). The PBAC's recommendation was based on, among other matters, its assessment that the cost effectiveness for Yonsa® MPRED™ would be acceptable if it was cost-minimised against the least costly treatment for mCRPC. The PBAC recommended flow-on changes to the restriction criteria of abiraterone (Zytiga®) would be required to prevent switching between the co-pack and individual components if disease progression occurred during treatment and to add a new caution regarding exercising caution in explaining correct dosing directions to the patients when changing between abiraterone products.
ACALABRUTINIB Capsule 100 mg Calquence® ASTRAZENECA PTY LTD Standard re-entry submission (Change to PBS listing) To be considered at a future PBAC meeting	Chronic lymphocytic leukaemia or small lymphocytic lymphoma	Resubmission to request a General Schedule Authority Required listing, for use in combination with obinutuzumab, for the treatment of previously untreated chronic lymphocytic leukaemia or small lymphocytic lymphoma in patients who are unsuitable for fludarabine-based chemoimmunotherapy.	Not Applicable	This item is to be considered at a future PBAC meeting.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
ADALIMUMAB Injection 20 mg in 0.2 mL pre-filled syringe 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 pen Injection 80 mg in 0.8 mL pre-filled syringe UPADACITINIB Tablet 15 mg Humira® Rinvoq® ABBVIE PTY LTD Category 3 submission (Change to PBS listing)	All adalimumab indications except juvenile idiopathic arthritis Severe active rheumatoid arthritis	To request that the authority levels for the current Humira listings be aligned with currently listed adalimumab biosimilars. To request that the upadacitinib listing for subsequent continuing treatment of severe active rheumatoid arthritis change from Authority Required (Written) to Authority Required (STREAMLINED).	Not Recommended	The PBAC did not recommend changes to the Authority Required levels of the Humira® brand of adalimumab to align with biosimilar brands for the requested indications and noted this recommendation aligns with the Government's biosimilar policies. The PBAC did not recommend changes to the Authority Required level for upadacitinib in the continuing phase listing for treatment of severe active rheumatoid arthritis to those recommended by the PBAC in March 2022 for other originator brands. The PBAC noted it was unable to determine whether the requested change would have a financial impact on the PBS based on the data provided in the submission. <u>Sponsor's Comment:</u> The sponsor had no comment.
ALIROCUMAB Injection 75 mg in 1 mL single use pre-filled pen Injection 150 mg in 1 mL single use pre-filled pen Praluent® SANOFI-AVENTIS AUSTRALIA PTY LTD Category 4 submission (Change to PBS listing) WITHDRAWN	Hypercholesterolaemia	To request an expansion of the current alirocumab listing to change to the low-density lipoprotein cholesterol criterion from 2.6 mmol/L to 1.8 mmol/L, and to allow general practitioners to initiate treatment in consultation with a specialist physician.	Not Applicable	This item was withdrawn.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
AMINO ACID FORMULA WITH VITAMINS AND MINERALS WITHOUT PHENYLALANINE Oral gel 85 g, 30 (PKU Squeezie) PKU Squeezie® VITAFLO AUSTRALIA PTY LIMITED Committee Secretariat submission (Change to PBS listing)	Phenylketonuria	To request that PKU Squeezie® with new source of vitamin A continue to be listed on the PBS under existing conditions.	Recommended	The PBAC recommended that PKU Squeezie® continue to be PBS-listed under the same conditions as currently listed. The PBAC considered the new source of vitamin A, retinyl palmitate, to be safe and appropriate.
AMINO ACID SYNTHETIC FORMULA SUPPLEMENTED WITH LONG CHAIN POLYUNSATURATED FATTY ACIDS, MEDIUM CHAIN TRIGLYCERIDES, 2'-FUCOSYLLACTOSE AND LACTO-N-NEOTETRAOSE Oral powder 400 g Alfamino® NESTLE AUSTRALIA LTD Category 3 submission (New PBS listing)	Cows' milk protein enteropathy Severe cows' milk protein enteropathy with failure to thrive Combined intolerance to cows' milk protein, soy protein and protein hydrolysate formulae Cows' milk anaphylaxis Proven combined immunoglobulin E (IgE) mediated allergy to cows' milk protein and soy protein Severe intestinal malabsorption including short bowel syndrome Eosinophilic oesophagitis	To request General Schedule Authority Required listing under the same conditions as the currently listed Alfamino®.	Recommended	The PBAC recommended the General Schedule Authority Required listing of amino acid synthetic formula supplemented with long chain polyunsaturated fatty acids, medium chain triglycerides, 2'-fucosyllactose and lacto-n-neotetraose (referred to as Alfamino® HMO) under the same conditions as the currently listed amino acid synthetic formula supplemented with long chain polyunsaturated fatty acids and medium chain triglycerides (referred to as Alfamino®). The PBAC's recommendation for listing was based on, among other matters, its assessment that the cost-effectiveness of Alfamino® HMO would be acceptable if it were cost-minimised to Alfamino®. The PBAC recommended that Alfamino® HMO be listed at the same price per gram of protein as Alfamino®.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
ASCIMINIB Tablet 20 mg Tablet 40 mg Scemblix® Novartis Pharmaceuticals Australia Pty Limited Early re-entry submission (New PBS listing)	Chronic myeloid leukaemia	Resubmission to request a General Schedule Authority Required listing for the treatment of patients with Philadelphia chromosome-positive chronic myeloid leukaemia in chronic phase previously treated with two or more tyrosine kinase inhibitors.	Recommended	The PBAC recommended the listing of asciminib for the treatment of (i) patients with Philadelphia chromosome-positive chronic myeloid leukaemia (Ph+ CML) in chronic phase (CP) or accelerated phase (AP), who had been previously treated with two or more tyrosine kinase inhibitors (TKIs); and (ii) patients with Ph+ CML in CP or AP, who had been previously treated with one or more TKIs and harbouring the T315I mutation. The PBAC considered that, despite the limitations of the clinical evidence, asciminib is non-inferior to nilotinib in patients without the T315I mutation, and non-inferior to ponatinib in patients with the T315I mutation. The PBAC considered that benchmarking against the second generation TKIs, nilotinib and dasatinib, in patients without the T315I mutation, was appropriate to establish a cost-effective price for asciminib, and an extension of this price to the very small number of patients who harbour the T315I mutation was acceptable. The PBAC considered that a written authority for T315I patients will be required. The PBAC recommended flow-on changes to the PBS restrictions for imatinib, dasatinib, nilotinib, and ponatinib, to add asciminib to the list of TKIs that can only be used as single agents at any one time and without concomitant interferon alfa therapy.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
AVATROMBOPAG Tablet 20 mg Doptelet® SWEDISH ORPHAN BIOVITRUM PTY LTD Category 2 submission (New PBS listing)	Chronic immune (idiopathic) thrombocytopenia purpura	To request a Section 100 (Highly Specialised Drugs Program) Authority Required (Written) listing for the treatment of severe thrombocytopenia in patients with chronic immune (idiopathic) thrombocytopenia purpura who have had an inadequate response or are intolerant to corticosteroids and intravenous immunoglobulin.	Recommended	The PBAC recommended the Section 100 (Highly Specialised Drugs Program – Public and Private Hospital), Authority Required listing of avatrombopag for the treatment of severe thrombocytopenia in patients with chronic idiopathic thrombocytopenic purpura (ITP). The PBAC's recommendation for listing was based on, among other matters, its assessment that the cost-effectiveness of avatrombopag would be acceptable if it were cost-minimised to eltrombopag. The PBAC considered there was uncertain but acceptable clinical evidence of non-inferiority to eltrombopag, and a moderate unmet clinical need for those patients for whom eltrombopag and romiplostim are unsuitable. The PBAC considered the equi-effective doses to be avatrombopag 22.34 mg daily = eltrombopag 55.2 mg daily. The PBAC recommended flow-on changes to the restriction criteria for eltrombopag and romiplostim would be required to allow for switching to avatrombopag.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
BUDESONIDE WITH GLYCOPYRRONIUM AND FORMOTEROL Pressurised inhalation containing budesonide 160 micrograms with glycopyrronium 7.2 micrograms and formoterol fumarate dihydrate 5 micrograms per dose, 120 doses Breztri Aerosphere® DFP-EvoCap ASTRAZENECA PTY LTD Category 4 submission (Change to PBS listing)	Chronic obstructive pulmonary disease	To request General Schedule Authority Required (STREAMLINED) listing for a new inhaler device under the same conditions as the current inhaler device.	Recommended	The PBAC held no objections to the planned replacement of the existing pressurised metered dose inhaler (pMDI) containing budesonide, glycopyrronium and formoterol (Breztri Aerosphere®) with a pMDI with a desiccated flow path (Breztri Aerosphere® DFP-EvoCap). The PBAC noted that the new Breztri Aerosphere® DFP-EvoCap would retain the same listing description as Breztri Aerosphere® and would therefore be listed under the same PBS item code as an additional brand. The PBAC advised, under Section 101 (4AACD) of the *National Health Act*, that Breztri Aerosphere® DFP- EvoCap and Breztri Aerosphere® should be considered equivalent for the purposes of substitution (i.e. a-flagged in the Schedule).

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
DAROLUTAMIDE Tablet 300 mg Nubeqa® BAYER AUSTRALIA LTD Category 2 submission (Change to PBS listing)	Prostate cancer	To request a General Schedule Authority Required listing, for use in combination with androgen deprivation therapy and docetaxel, for the treatment of metastatic hormone sensitive prostate cancer.	Deferred	The PBAC deferred its decision on whether to recommend darolutamide for the treatment of mHSPC. The PBAC was of a mind to recommend darolutamide, pending advice from the TGA Delegate and, in the circumstance where apalutamide is not PBS-listed for mHSPC, revised financials estimates and parameters for a risk sharing arrangement (RSA) are provided. The PBAC noted the submission's proposed listing was for darolutamide in combination with docetaxel and androgen deprivation therapy (ADT), i.e. triple therapy only. However, the PBAC considered that, in order to increase clinical choice, the PBS restriction for darolutamide should mirror that previously recommended for apalutamide and allow use as dual therapy in combination with ADT or as triple therapy in combination with docetaxel and ADT. The PBAC considered that darolutamide, in combination with docetaxel and ADT, provides a moderate clinical benefit for patients with mHSPC compared with docetaxel plus ADT. The PBAC advised that changes should be made to the economic model and that for darolutamide to be considered cost-effective, the price should be reduced such that the incremental cost-effectiveness ratio is no more than $35,000 to < $45,000 per quality adjusted life year gained, which was consistent with previous PBAC recommendations for this condition. The PBAC considered that uncertainties in the utilisation estimates could be managed by an RSA. <u>Sponsor's Comment:</u> Bayer will continue to work with the PBAC's deferral decision to enable earliest possible access to darolutamide for mHSPC patients.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
DEUCRAVACITINIB Tablet 6 mg Sotyktu® BRISTOL-MYERS SQUIBB AUSTRALIA PTY LTD Category 1 submission (New PBS listing)	Plaque psoriasis	To request a General Schedule Authority Required (STREAMLINED) listing for the treatment of severe chronic plaque psoriasis, in patients who have not responded to, or have a contraindication or demonstrated intolerance to methotrexate.	Deferred	The PBAC deferred making a recommendation for the listing of deucravacitinib for the treatment of severe chronic plaque psoriasis, to seek additional expert clinical advice on its appropriate and likely place in therapy. In deferring the matter, the PBAC was uncertain as to whether the proposed clinical place of deucravacitinib, in the same line of therapy as apremilast, i.e. non- or inadequate response/intolerance/contraindication to methotrexate, but prior to treatment with biologics was reflective of its appropriate use in practice, and whether there was potentially a substantial overlap with the population eligible for biologics. The PBAC requested further dermatology expert clinical advice to resolve this uncertainty, as the cost-effectiveness proposition of deucravacitinib was dependent on the likely place in therapy and most appropriate comparator selection. <u>Sponsor's Comment:</u> The Sponsor is committed to working with the PBAC to bring deucravacitinib for the treatment of chronic plaque psoriasis to Australian patients in a timely manner.

DOSTARLIMAB Solution concentrate for I.V. infusion 500 mg in 10 mL Jemperli® GLAXOSMITHKLINE AUSTRALIA PTY LTD Standard re-entry submission (New PBS listing)	Endometrial cancer	Resubmission to request a Section 100 (Efficient Funding of Chemotherapy Program) Authority Required (STREAMLINED) listing for the treatment of recurrent or advanced mismatch repair deficient (dMMR) endometrial cancer that has progressed on or following prior treatment with a platinum-containing regimen.	Not Recommended	The resubmission requested a Section 100 listing for dostarlimab for the treatment of recurrent or advanced dMMR endometrial cancer that has progressed on or following prior treatment with a platinum-containing regimen. <u>Comparator:</u> Standard of care (SoC) comprising single-agent chemotherapy and platinum-based chemotherapy (PBC) was nominated as the main comparator. The resubmission also nominated pembrolizumab in combination with lenvatinib (PEM+LEN) and pembrolizumab monotherapy as near market comparators. The PBAC considered that SoC comprising single-agent chemotherapy and PBC was appropriate as the main comparator. The PBAC considered PEM+LEN was an appropriate near-market comparator, but noted it was not PBS-listed at the time of PBAC consideration <u>Clinical claim:</u> Superior efficacy and non-inferior safety compared to SoC. The PBAC considered that the claim of superior comparative effectiveness versus SoC was reasonable, but the magnitude of the incremental benefit versus SoC was unable to be reliably estimated. The PBAC considered that the claim of non-inferior comparative safety versus SoC was reasonable. Non-inferior comparative effectiveness and superior safety compared with PEM+LEN. The PBAC considered that the claim of non-inferior comparative effectiveness versus PEM+LEN was not adequately supported by the data given the high risk of bias in the comparison, and the immaturity of the overall survival data. The PBAC considered that the

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				claim of superior comparative safety versus PEM+LEN was likely to be reasonable. <u>Economic claim:</u> Cost-utility analysis versus SoC. The PBAC considered that the cost-effectiveness of dostarlimab versus SoC was unable to be reliably assessed due to the uncertain magnitude of benefit for dostarlimab, based on data from a relatively small single arm study. Cost-minimisation approach versus PEM+LEN. The PBAC considered that the cost-minimisation approach versus PEM+LEN was not informative as non-inferiority had not been adequately established. <u>Sponsor's Comment:</u> The sponsor had no comment.
DUPILUMAB Injection 300 mg in 2 mL single dose autoinjector Injection 200 mg in 1.14 mL single dose autoinjector Dupixent® SANOFI-AVENTIS AUSTRALIA PTY LTD Category 4 submission (Change to PBS listing)	Chronic severe atopic dermatitis Uncontrolled severe asthma	To request a General Schedule Authority Required listing for chronic severe atopic dermatitis and a Section 100 (Highly Specialised Drugs Program) Authority Required listing for uncontrolled severe asthma of two new forms under the same conditions as the currently listed forms of dupilumab.	Recommended	The PBAC recommended the listing of two new forms of dupilumab: 200 mg in 1.14 mL and 300 mg in 2 mL single dose autoinjector (AI) under the same circumstances as the currently listed dupilumab 200 mg in 1.14 mL and 300 mg in 2 mL single dose pre-filled syringe (PFS). The PBAC recommended the listing on a cost-minimisation basis compared to the relevant lowest-cost comparator. The PBAC noted that the submission had estimated the following equi-effective doses and considered that they were appropriate: 200 mg in 1.14 mL dupilumab AI = 200 mg in 1.14 mL dupilumab PFS; 300 mg in 2 mL dupilumab AI = 300 mg in 2 mL dupilumab PFS. The PBAC considered that, under Section 101(4AACD) of the *National Health Act 1953*, the AI and PFS forms of the same strength of dupilumab should be considered equivalent for the purposes of substitution (i.e., 'a'-flagged to each other in the Schedule).

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
ELEXACAFTOR WITH TEZACAFTOR AND WITH IVACAFTOR, AND IVACAFTOR Pack containing 56 tablets elexacaftor 100 mg with tezacaftor 50 mg and with ivacaftor 75 mg and 28 tablets ivacaftor 150 mg Pack containing 56 tablets elexacaftor 50 mg with tezacaftor 25 mg and with ivacaftor 37.5 mg and 28 tablets ivacaftor 75 mg Trikafta® VERTEX PHARMACEUTICALS (AUSTRALIA) PTY. LTD. Category 2 submission (New PBS listing)	Cystic fibrosis	To request a Section 100 (Highly Specialised Drugs Program) Authority Required listing for the treatment of cystic fibrosis in patients who are aged 6 to 11 years and who have at least one F508del mutation on the cystic fibrosis transmembrane conductance regulator (CFTR) gene.	Recommended	The PBAC recommended elexacaftor/ tezacaftor/ ivacaftor (ELZ/TEZ/IVA) for the treatment of cystic fibrosis in patients who are aged 6 to 11 years and who have at least one F508del mutation on the CFTR gene. The PBAC noted the evidence presented could not accurately quantify the benefit of treating patients with ELX/TEZ/IVA from a younger age but acknowledged treatment from a young age was likely to be beneficial. The PBAC considered ELX/TEZ/IVA was likely to be cost-effective for this population at a unit price no higher than that of the current PBS listing (for patients over 12 years of age) as proposed by the sponsor in its pre-PBAC response. The PBAC considered a number of assumptions applied in the financial estimates were overly optimistic and should be reduced to be consistent with the assumptions applied to the financial estimates agreed for the population of patients over 12 years of age.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
EMPAGLIFLOZIN Tablet 10 mg Jardiance® BOEHRINGER INGELHEIM PTY LTD Category 2 submission (Change to PBS listing)	Chronic heart failure	To request a General Schedule Authority Required (STREAMLINED) listing for the treatment of chronic heart failure (NYHA classes II, III or IV) in patients with a left ventricular ejection fraction greater than 40%.	Not Recommended	The PBAC did not recommend empagliflozin for the treatment of chronic heart failure in patients with a left ventricular ejection fraction greater than 40%. The PBAC considered there was a high unmet clinical need for effective treatments for patients with this condition. The PBAC noted that empagliflozin when added to standard care provided a statistically significant improvement in efficacy over standard care alone in the proposed population. However, the reduction in hospitalisations due to heart failure were modest and there remained some uncertainty in the extent of any mortality benefit. The PBAC noted the economic model did not adequately capture the progressive nature of the disease. The PBAC considered that a price reduction to achieve a lower incremental cost-effectiveness ratio was required to reflect the moderate clinical benefits shown in the clinical evidence and the uncertain extrapolated benefit. The PBAC considered that a risk sharing arrangement would be required due to a high risk of utilisation outside the proposed restriction. The PBAC nominated the Early Re-entry re-submission pathway for this item. <u>Sponsor's Comment:</u> The sponsor had no comment.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
ENFORTUMAB VEDOTIN Powder for I.V. infusion 20 mg Powder for I.V. infusion 30 mg Padcev® Astellas Pharma Australia Pty Ltd Early re-entry submission (New PBS listing)	Urothelial cancer	Resubmission to request a Section 100 (Efficient Funding of Chemotherapy Program) Authority Required (STREAMLINED) listing for the treatment of patients with locally advanced (Stage III) or metastatic (Stage IV) urothelial cancer 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.	Deferred	The PBAC deferred making a recommendation for 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 PD-1 or a PD-L1 inhibitor to allow further consultation with the sponsor regarding the approach to achieving an acceptable incremental cost-effectiveness ratio. <u>Sponsor's Comment:</u> Astellas Australia looks forward to progressing through the PBAC processes to enable enfortumab vedotin to be available on the PBS for suitable patients with locally advanced (Stage III) or metastatic (Stage IV) urothelial cancer.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
ETANERCEPT Injection 50 mg in 1 mL single use auto-injector, 4 Nepexto® ALPHAPHARM PTY LTD Category 3 submission (New PBS listing)	Rheumatoid arthritis Juvenile idiopathic arthritis Psoriatic arthritis Plaque psoriasis Ankylosing spondylitis	To request General Schedule Authority Required listing of an etanercept biosimilar under the same conditions as its reference biologic.	Recommended	The PBAC recommended the Authority Required listing of etanercept (Nepexto®) in the form of injection 50 mg in 1 mL single use auto-injector as a biosimilar brand of Enbrel® on the General Schedule and Section 100 (Highly Specialised Drug Program) for the same indications as Enbrel® 50 mg in 1 mL single use auto-injector. The PBAC's recommendation for listing was based on, among other matters, its assessment that the cost-effectiveness of Nepexto® would be acceptable if it were cost-minimised to Enbrel®. The PBAC noted the listing of Nepexto® is expected to have no change in the overall net cost to the government. The PBAC advised the equi-effective doses are Nepexto® 50 mg = Enbrel® 50 mg = Brenzys® 50 mg. The PBAC advised that, the same strengths of Enbrel®, Brenzys®, Rymti® (if listed) and Nepexto® in all forms should be treated as equivalent to each other for the purpose of substitution. The PBAC considered that biosimilar uptake drivers should apply to Nepexto® consistent with the current PBS listings for etanercept biosimilar Brenzys®. The PBAC advised the addition of the following caution to Nepexto® and flow-on restriction to relevant indications/products for Enbrel® to align with the TGA Product Information: 'This formulation of etanercept is intended for use in children and adolescents weighing 62.5 kg or more.'

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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 Solution for injection 225 mg in 1.5 mL single dose pre-filled syringe Solution for injection 225 mg in 1.5 mL single dose autoinjector Ajovy® TEVA PHARMA AUSTRALIA PTY LTD Category 2 submission (Change to PBS listing)	Migraine	To request a General Schedule Authority Required (STREAMLINED) listing for the treatment of high frequency episodic migraine (HFEM) in patients who have had an inadequate response, intolerance or a contraindication to at least three prophylactic migraine medications.	Recommended	The PBAC recommended amending the current listing of fremanezumab for chronic migraine to include the treatment of patients with treatment-resistant HFEM. Consistent with its March 2022 recommendation for galcanezumab in this population, the PBAC considered fremanezumab would be cost-effective for the HFEM population at a price no higher than the effective price for fremanezumab for patients with chronic migraine. The PBAC considered that the treatment-resistant HFEM population should be included in the current risk sharing arrangement (RSA) in place for chronic migraine. The PBAC noted that patients may move between these categories and considered that including the HFEM population in the RSA would help to manage the risk of use in a broader population where treatment may be less cost-effective. The PBAC recommended minor editorial flow-on changes to the PBS restrictions for other listed or recommended migraine drugs (galcanezumab, eptinezumab).
GLYCOMACROPEPTIDE FORMULA WITH LONG CHAIN POLYUNSATURATED FATTY ACIDS AND DOCOSAHEXAENOIC ACID AND LOW IN PHENYLALANINE Oral liquid, 237 mL, 15 (PKU Sphere Liquid) PKU Sphere Liquid VITAFLO AUSTRALIA PTY LIMITED Category 3 submission (New PBS listing)	Phenylketonuria	To request a General Schedule Restricted Benefit listing for the dietary management of patients with phenylketonuria.	Recommended	The PBAC recommended the General Schedule Restricted Benefit listing of glycomacropeptide formula with long chain polyunsaturated fatty acids and docosahexaenoic acid and low in phenylalanine, bottle containing oral liquid, 237 mL (PKU Sphere Liquid) for the dietary management of patients with phenylketonuria under the same circumstances as, and on a cost-minimisation basis to, PKU Sphere20® Powder at the same price per gram protein equivalent.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
IBRUTINIB Capsule 140 mg Imbruvica® JANSSEN-CILAG PTY LTD Category 2 submission (Change to PBS listing) To be considered at a future PBAC meeting	Chronic lymphocytic leukaemia or small lymphocytic lymphoma	To request a General Schedule Authority Required (Written) listing, for use in combination with venetoclax, for the treatment of previously untreated chronic lymphocytic leukaemia or small lymphocytic lymphoma.	Not Applicable	This item is to be considered at a future PBAC meeting.
INFLIXIMAB Solution for injection 120 mg in 1 mL pre-filled pen Solution for injection 120 mg in 1 mL pre-filled syringe Remsima® SC CELLTRION HEALTHCARE AUSTRALIA PTY LTD Category 3 submission (Change to PBS listing)	Rheumatoid arthritis	To request a Section 100 (Highly Specialised Drugs Program) Authority Required listing for initial treatment of severe active rheumatoid arthritis.	Recommended	The PBAC recommended a new Authority Required listing that provides loading doses for infliximab (Remsima® SC) for the treatment of severe active rheumatoid arthritis whereby 120 mg is given subcutaneously at weeks 0, 1, 2, 3 and 4, and then every 2 weeks thereafter. The PBAC's recommendation was made on the basis that, among other matters, it should be listed on a cost-minimisation basis with the lowest cost alternative treatment, calculated over 2 years at the ex-manufacturer price.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
LANREOTIDE Injection 60 mg (as acetate) in single dose pre-filled syringe Injection 90 mg (as acetate) in single dose pre-filled syringe Injection 120 mg (as acetate) in single dose pre-filled syringe Mytolac® AMDIPHARM MERCURY (AUSTRALIA) PTY LIMITED Category 3 submission (New PBS listing)	Acromegaly Functional carcinoid tumour Non-functional gastroenteropancreatic neuroendocrine tumour (GEP-NET)	To request Section 100 (Highly Specialised Drugs Program) Authority Required (STREAMLINED) listing of a new pre-filled syringe under the same conditions as the current pre-filled syringe.	Recommended	The PBAC recommended the Authority Required listing of lanreotide (Mytolac®) 60 mg, 90 mg and 120 mg (as acetate) in pre-filled, single use syringe as part of the Section 100 (Highly Specialised Drug Program) under the same circumstances as the PBS-listed reference brand Somatuline Autogel®. The PBAC's recommendation for listing was based on, among other matters, its assessment that the cost-effectiveness of Mytolac® would be acceptable if it were cost-minimised to Somatuline Autogel® for the same indications. In making this recommendation, the PBAC noted concerns raised by the Consumer Comments regarding quality use of medicines but considered that the training and support programs provided by the sponsor should adequately address these concerns. The PBAC therefore considered that Mytolac® and Somatuline Autogel® be considered equivalent for the purposes of substitution. The PBAC recommended flow-on changes to the PBS listings for Somatuline Autogel® to note that patients should be educated regarding the correct administration of the injection upon dispensing.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
LENACAPAVIR Tablet 300 mg Pack containing 2 injection sets 463.5 mg in 1.5 mL Sunlenca® GILEAD SCIENCES PTY LIMITED Category 1 submission (New PBS listing)	Human immunodeficiency virus infection	To request both a Section 100 (Highly Specialised Drugs Program) and Section 100 (Highly Specialised Drugs Program – Community Access) Authority Required (STREAMLINED) listing for the treatment of patients with highly multi-drug resistant human immunodeficiency virus type 1 infection.	Not Recommended	The PBAC did not recommend the listing of lenacapavir for the treatment of hMDR HIV-1 infection. The PBAC considered that the composition of the nominated comparator of optimised background regimen (OBR) was not reflective of contemporary Australian practice and therefore the comparative effectiveness of lenacapavir + OBR to OBR alone was uncertain, although noted lenacapavir appears to be effective for some patients for the treatment of HIV-1 infection when used in combination with OBR. The PBAC considered that the economic model was highly uncertain due to the extremely limited amount of data to inform the transitions between a large number of health states. Overall, the PBAC considered the economic model was largely uninformative and the incremental cost-effectiveness ratio unacceptably high at the proposed price. <u>Sponsor's Comment:</u> Gilead is disappointed by the decision however acknowledges the PBAC's recognition of the high unmet clinical need for the treatment of people with HIV who are highly multidrug resistant. Gilead will continue to seek to gain PBAC recommendation and PBS listing to ensure that people living with HIV who are highly multidrug resistant have access to a much-needed new treatment option.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
MAVACAMTEN Capsule 2.5 mg Capsule 5 mg Capsule 10 mg Capsule 15 mg Camzyos® BRISTOL-MYERS SQUIBB AUSTRALIA PTY LTD Category 1 submission (New PBS listing)	Hypertrophic cardiomyopathy	To request a General Schedule Authority Required listing for the treatment of adults with symptomatic obstructive hypertrophic cardiomyopathy (HCM).	Not Recommended	The PBAC did not recommend the listing of mavacamten for the treatment of adults with symptomatic obstructive HCM. The PBAC considered it is likely that mavacamten with or without beta-blocker (BB)/calcium channel blocker (CCB) provided a short-term, moderate clinical benefit over standard of care (BB/CCB), in terms of symptomatic improvement, but the longer-term clinical benefit and safety and the impact on other patient-relevant clinical endpoints such as hospitalisations or mortality is unknown. The PBAC considered that the model relied on highly uncertain and optimistic assumptions regarding the long-term clinical benefit and the incremental cost-effectiveness ratio presented in the submission was underestimated. <u>Sponsor's Comment:</u> The Sponsor is committed to working with the PBAC to bring mavacamten for the treatment of adults with symptomatic obstructive HCM to Australian patients in a timely manner.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
MEPOLIZUMAB Injection 100 mg in 1 mL single dose pre-filled pen Nucala® GLAXOSMITHKLINE AUSTRALIA PTY LTD Standard re-entry submission (Change to PBS listing)	Chronic rhinosinusitis with nasal polyps	Resubmission to request a Section 100 (Highly Specialised Drugs Program) Authority Required (Written) listing for the treatment of chronic rhinosinusitis with nasal polyps.	Recommended	The PBAC recommended the Authority Required listing of mepolizumab for the treatment of chronic rhinosinusitis with nasal polyps, on the basis that it should be available only under special arrangements under Section 100 (Highly Specialised Drugs Program). The PBAC considered that the blood eosinophil count threshold for access to mepolizumab should be ≥300 cells/µL. The PBAC is satisfied that mepolizumab provides, for some patients, a significant improvement in efficacy over standard of care. The PBAC considered that due to the limitations of the treatment options currently available, the addition of mepolizumab offered high added therapeutic value. The PBAC's recommendation for listing was based on, among other matters, its assessment, that the cost-effectiveness of mepolizumab would be acceptable at the price proposed in the pre-PBAC response, and with a risk sharing arrangement to address the uncertainty associated with including patients unsuitable for surgery in the proposed PBS population.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
MIDAZOLAM Oromucosal solution in pre-filled syringe 2.5 mg in 0.25 mL Oromucosal solution in pre-filled syringe 5 mg in 0.5 mL Oromucosal solution in pre-filled syringe 7.5 mg in 0.75 mL Oromucosal solution in pre-filled syringe 10 mg in 1 mL Zyamis® Clinect Pty Ltd Early re-entry submission (New PBS listing)	Epilepsy	Resubmission to request a General Schedule Authority Required listing for the treatment of generalised convulsive status epilepticus in patients with epilepsy aged over 6 months and a high risk of status epilepticus.	Recommended	The PBAC recommended the listing of midazolam oromucosal solution in pre-filled syringes (2.5 mg, 5 mg, 7.5 mg and 10 mg), for the treatment of generalised convulsive status epilepticus (GCSE) in patients aged over 6 months. The PBAC noted that GCSE is a stressful situation for parents and carers, and considered that the proposed listing, incorporating expert clinical advice, offered clinically meaningful benefits by improved quality use of medicines with easier and more accurate administration. This is consistent with stakeholder comments which were received from health professionals, individuals and organisations. The PBAC's recommendation for listing was based on, among other matters, its assessment that the cost-effectiveness of midazolam oromucosal solution in pre-filled syringes would be acceptable if it were cost-minimised against off-label use of midazolam ampoules. However, the PBAC recognised there were additional health outcome benefits to this formulation associated with the accurate and timely administration of midazolam during an acute health emergency that justified a significant price premium under the specific circumstances of the restriction. The PBAC considered that the resubmission had partly addressed the concerns raised at the July 2022 PBAC meeting, and the remaining concerns could be addressed with an appropriate risk sharing arrangement.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
MOBOCERTINIB Capsule 40 mg Exkivity® TAKEDA PHARMACEUTICALS AUSTRALIA PTY. LTD. Category 1 submission (New PBS listing)	Non-small cell lung cancer	To request a General Schedule Authority Required listing for the treatment of adults with epidermal growth factor receptor (*EGFR*) exon 20 insertion positive locally advanced or metastatic (Stage IIIB/IV) non-small cell lung cancer who have received platinum-based chemotherapy.	Not Recommended	The PBAC did not recommend mobocertinib for the treatment of adults with *EGFR* exon 20 insertion positive locally advanced or metastatic (Stage IIIB/IV) non-small cell lung cancer (NSCLC) who have received platinum-based chemotherapy. The PBAC considered the nominated comparator of Standard of Care, comprising EGFR tyrosine kinase inhibitors (TKIs), immune checkpoint inhibitor (ICI) monotherapy, and chemotherapy was inappropriate as EGFR TKI and ICI are generally not used in these patients. The PBAC considered that the evidence presented is associated with a very high degree of uncertainty and did not permit either a comparison of mobocertinib with purely chemotherapy-based treatments, or a comparison of safety. <u>Sponsor's Comment:</u> Takeda acknowledges the limited evidence base available for mobocertinib which has been listed with provisional registration in Australia. The Sponsor is committed to working with the PBAC to bring this new medicine for a rare subtype of lung cancer to Australian patients in a timely manner.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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 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 Early re-entry submission (Change to PBS listing)	Oesophageal carcinoma or gastroesophageal junction carcinoma	Resubmission to request a Section 100 (Efficient Funding of Chemotherapy Program) Authority Required listing for the adjuvant treatment of patients with oesophageal carcinoma or gastroesophageal junction carcinoma who have previously received platinum-based chemoradiotherapy and surgery.	Recommended	The PBAC recommended the Section 100 (Efficient Funding of Chemotherapy Program) Authority Required listing of nivolumab for the adjuvant treatment of patients with oesophageal cancer or gastroesophageal junction cancer who have received platinum-based chemoradiotherapy and surgery. The PBAC considered that the resubmission had addressed the substantive outstanding issues identified at the July 2022 PBAC meeting. However, the PBAC noted that additional revisions were made to the economic model that were not included in the early re-entry pathway nominated at the July 2022 PBAC meeting and noted that the additional changes made to the economic model favoured nivolumab. The PBAC therefore considered a further price reduction would be required to achieve a cost-effective listing for adjuvant nivolumab. The PBAC advised the net cost of listing nivolumab in the adjuvant treatment setting should be revised to account for the reduced use of immunotherapies in the advanced/metastatic treatment setting. The PBAC recommended flow-on restriction changes would be required to immunotherapies listed for advanced/metastatic gastro-oesophageal cancers to reflect one treatment course per patient per lifetime.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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 plus IPILIMUMAB NIVOLUMAB: Injection concentrate for I.V. infusion 100 mg in 10 mL, Injection concentrate for I.V. infusion 40 mg in 4 mL IPILIMUMAB: Injection concentrate for I.V. infusion 200 mg in 40 mL, Injection concentrate for I.V. infusion 50 mg in 10 mL Opdivo® Yervoy® BRISTOL-MYERS SQUIBB AUSTRALIA PTY LTD Category 2 submission (Change to PBS listing)	Melanoma	To request an expansion of the current nivolumab plus ipilimumab listing to allow patients who experience disease recurrence either while receiving or within 6 months of completing adjuvant treatment with an anti-programmed death-1 (PD-1) based therapy to receive nivolumab plus ipilimumab in the metastatic setting.	Recommended	The PBAC recommended expanding the listing of nivolumab in combination with ipilimumab (NIVO + IPI) to allow the treatment of Stage III or IV malignant melanoma in patients who experience melanoma recurrence while receiving or within 6 months of completing adjuvant PD-1 inhibitor monotherapy. The PBAC noted that the magnitude of benefit of NIVO + IPI in the proposed population was highly uncertain due to the low quality of the clinical evidence presented. The PBAC considered that, although uncertain, the cost-effectiveness of NIVO + IPI as previously determined for patients with unresectable Stage III or IV malignant melanoma was unlikely to be substantially altered by inclusion of the expanded population. The PBAC considered these uncertainties were acceptable in the context of the modest financial impact and advised that the financial impact be managed through the existing PD-1 inhibitor melanoma risk sharing arrangement.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
OLAPARIB Tablet 100 mg Tablet 150 mg Lynparza® AstraZeneca Pty Ltd Early re-entry submission (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 BRCA wild type advanced epithelial ovarian, fallopian tube or primary peritoneal cancer.	Deferred	The PBAC deferred its decision on whether to recommend olaparib for maintenance therapy in patients with newly diagnosed HRD positive BRCA wild type advanced epithelial ovarian, fallopian tube or primary peritoneal cancer. The PBAC was of a mind to recommend olaparib pending MSAC consideration of HRD testing. The PBAC noted that MSAC did not support public funding of HRD testing for access to olaparib during its consideration in July 2022 and that a resubmission for MBS funding had been lodged by the sponsor. The PBAC noted the new data provided in the Early Re-entry resubmission and considered that the resubmission was largely consistent with the changes requested by the PBAC at its July 2022 meeting. The PBAC considered that a further price reduction would be required to ensure cost-effectiveness given the remaining uncertainty in the modelled benefit of olaparib in the proposed population. The PBAC noted that uncertainties remained with respect to the proposed HRD testing which would require MSAC advice. <u>Sponsor's Comment:</u> The sponsor had no comment.

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ONASEMNOGENE ABEPARVOVEC Solution for injection, customised based on patient weight Zolgensma® NOVARTIS PHARMACEUTICALS AUSTRALIA PTY LIMITED Category 2 submission (Change to PBS listing)	Spinal muscular atrophy	To request an expansion of the current onasemnogene abeparvovec listing to include the presymptomatic treatment of children with 3 copies of the Survival Motor Neuron 2 (*SMN2*) gene with spinal muscular atrophy.	Not Recommended	The PBAC did not recommend onasemnogene abeparvovec (ONA) (Zolgensma®) for the treatment of pre-symptomatic patients who are genetically diagnosed with spinal muscular atrophy (SMA) and have 3 copies of the *SMN2* gene. The PBAC considered that pre-symptomatic treatment with ONA may provide clinical benefit for some patients compared to treatment with existing disease modifying therapies following the onset of symptoms. However, the PBAC noted the magnitude of benefit was unclear from the limited clinical data available. The PBAC considered that the economic model presented was unreliable for informing the cost-effectiveness of ONA. The PBAC noted that the price requested was substantially higher than the current price for patients with 1-2 copies of *SMN2* and considered this to be inconsistent with the reduced incremental benefit that would be observed for patients with 3 copies of *SMN2*. The PBAC considered for ONA to be similarly cost effective in the expanded population that the price of ONA would need to be substantially less than for the current listing. <u>Sponsor's Comment:</u> Zolgensma® has demonstrated significant impact on the lives of SMA babies with 3 *SMN2* copies and has the potential to fundamentally change the quality of life of these Australians, with a single dose treatment. Novartis appreciates that the PBAC has recognised this clinical benefit. With newborn screening becoming more available in Australia, Novartis will continue to work with PBAC in the hope that this treatment will be available to all diagnosed presymptomatic SMA babies.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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 Capsule 920 micrograms Pack containing 4 capsules 230 micrograms and 3 capsules 460 micrograms Zeposia® CELGENE PTY LIMITED Category 2 submission (Other matters)	Ulcerative colitis	To request the PBAC consider its previous recommendation to list ozanimod for the treatment of moderate to severe ulcerative colitis (MSUC) on a cost-minimisation basis with the least costly alternative disease-modifying anti-rheumatic drug.	Recommended	The PBAC revised its March 2022 recommendation of a General Schedule, Authority Required listing of ozanimod (OZA) for the treatment of MSUC, to specify that OZA should be listed on a cost-minimisation with the least costly alternative biologic or targeted synthetic disease modifying anti-rheumatic drug (bDMARD/tsDMARD) listed for MSUC, excluding adalimumab (ADA). In revising its recommendation, the PBAC considered additional evidence including a matching-adjusted indirect comparison, and network meta-analysis, which it considered were methodologically sound and reliable for assessing a claim that OZA provides, for some patients, a significant improvement in effectiveness over ADA. The PBAC considered the additional analyses supported such a claim and therefore revised its March 2022 recommendation to specify that ADA should not be considered as part of the cost-minimisation to the least costly alternative bDMARD/tsDMARD. The PBAC noted a claim of superior comparative effectiveness was also made versus golimumab (GOL), however considered the evidence presented did not adequately support a claim of superiority over GOL.

PATIROMER Sachet, 8.4 g powder for oral liquid Sachet, 16.8 g powder for oral liquid Veltassa® VIFOR PHARMA PTY LIMITED Standard re-entry submission (New PBS listing)	Hyperkalaemia	Resubmission to request a General Schedule Authority Required listing for the treatment of chronic hyperkalaemia in patients with stage 3 or stage 4 chronic kidney disease (CKD).	Not Recommended	The PBAC did not recommend the listing of patiromer (Veltassa®) for the treatment of hyperkalaemia in patients with CKD Stage 3 or 4 and chronic hyperkalaemia who are receiving or intolerant to a renin angiotensin aldosterone system inhibitor (RAASi). The PBAC noted that the additional clinical evidence presented in the resubmission did not address the uncertainties surrounding the long-term benefits of patiromer. The PBAC considered that the cost-utility analysis was unreliable for decision making and relied on long-term outcomes that were not supported by the clinical data. The PBAC advised that it would be more appropriate for the price of patiromer to be based on a cost-minimisation approach versus sodium polystyrene sulfonate (SPS) or calcium polystyrene sulfonate (CPS) resins given that SPS/CPS resins could be used to treat the overall target population. The PBAC recommended the early re-entry pathway for this item. Comparator: Standard care was nominated as the main comparator. SPS and CPS resins were nominated as secondary comparators. The PBAC considered that SPS and CPS resins were appropriate comparators. Clinical claim vs standard of care: Superior efficacy compared to standard care alone. Inferior safety compared to standard care alone. The PBAC considered that the claim of superior comparative effectiveness was adequately supported for the outcome of potassium lowering, however it remained unknown whether patiromer confers a clinically important difference in terms of optimisation/maintenance of RAASi treatment and long-term cardiovascular and renal outcomes. The PBAC considered that the claim of inferior comparative safety versus standard care was reasonable.

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				Clinical claim vs SPS/CPS resins: Non-inferior efficacy compared to SPS/CPS resins. Non-inferior safety compared to SPS/CPS resins. The PBAC considered that the claim that patiromer was non-inferior in terms of reducing potassium levels compared to SPS/CPS resins was likely reasonable. The PBAC considered that the claim of non-inferior comparative safety versus SPS/CPS resins was reasonable and considered that patiromer may be associated with fewer gastrointestinal adverse events. Economic claim vs standard of care: Cost-effectiveness analysis versus standard care (placebo). The PBAC considered the economic model was overly complex and unreliable for decision making due to inconsistencies, double counting and systematic errors. The PBAC also considered that a number of assumptions included in the model were highly uncertain and favoured patiromer. These included, the implausibly high rate of RAASi discontinuation in the placebo arm, the assumption that short-term patiromer treatment would result in prolonged RAASi therapy and the long-term gain of cardiovascular and renal benefits, the assumption that all patients would be receiving maximal RAASi therapy at baseline and the low assumed doses of patiromer. Economic claim vs SPS/CPS resins: Cost-minimisation approach versus SPS/CPS resins. The PBAC noted that the cost-minimisation approach incorrectly included patients with both acute and chronic hyperkalaemia which was inconsistent with the proposed patiromer restriction. Sponsor's Comment: The sponsor had no comment.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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 Solution concentrate for I.V. infusion 100 mg in 4 mL Keytruda® MERCK SHARP & DOHME (AUSTRALIA) PTY LTD Category 2 submission (Change to PBS listing)	Cervical cancer	To request a Section 100 (Efficient Funding of Chemotherapy Program) Authority Required listing for the treatment of persistent, recurrent or metastatic (stage IVB) squamous cell carcinoma, adenocarcimoma and adenosquamous carcinoma of the cervix in patients whose tumours express programmed cell death ligand-1 (PD-L1) combined positive score (CPS) equal to or greater than 1.	Recommended	The PBAC recommended the listing of pembrolizumab for the treatment of patients with persistent, recurrent, or metastatic (Stage IVB) cervical cancer. The PBAC noted that although rates of cervical cancer are declining, there remains a high clinical need for effective treatment in this patient population, particularly for communities disproportionately affected by cervical cancer. The PBAC was satisfied that pembrolizumab in combination with chemotherapy provides a meaningful improvement in overall survival, compared with standard chemotherapy alone. Although the submission proposed treatment in patients with PD-L1 CPS ≥1, the PBAC noted that the survival benefit was demonstrated in the full trial population and therefore recommended listing without restriction based on PD-L1 status. The PBAC considered that pembrolizumab would be cost-effective with a price reduction.

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PEMBROLIZUMAB Solution concentrate for I.V. infusion 100 mg in 4 mL Keytruda® MERCK SHARP & DOHME (AUSTRALIA) PTY LTD Category 3 submission (Other matters)	Locally advanced (Stage III) or metastatic (Stage IV) urothelial cancer	To request the PBAC consider the previously estimated utilisation for locally advanced or metastatic urothelial cancer.	Not Recommended	The PBAC did not advise that it previous recommendation regarding the estimated utilisation for locally advanced (Stage III) or metastatic urothelial (Stage IV) cancer be amended. The PBAC noted the listing of avelumab as first-line maintenance treatment for urothelial cancer is likely to continue to reduce the uptake rate of second-line pembrolizumab steadily because restrictions for pembrolizumab prevent sequential use following treatment with avelumab. <u>Sponsor's Comment:</u> MSD is disappointed that PBAC has not recommended an amendment to the estimated utilisation for urothelial cancer given the independent DUSC review in Oct 2021 showed that the patient numbers were underestimated, with minimal leakage and shorter than anticipated time on treatment. MSD is of the view that the listing of avelumab is unlikely to significantly reduce the uptake rate of pembrolizumab, therefore presenting an ongoing risk to MSD. MSD will continue to assess what available options exist to support an appropriate amendment to the estimated utilisation of pembrolizumab.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
PNEUMOCOCCAL POLYSACCHARIDE CONJUGATE VACCINE, 20-VALENT ADSORBED 0.5 mL pre-filled syringe Prevenar 20® PFIZER AUSTRALIA PTY LTD Category 2 submission (New NIP listing)	Prevention of pneumococcal disease	To request a National Immunisation Program listing for the prevention of pneumococcal disease.	Recommended	The PBAC recommended that 20-valent pneumococcal conjugate vaccine (20vPCV, Prevenar 20®) be a designated vaccine for the purposes of the *National Health Act 1953*, for the prevention of pneumococcal disease in individuals with an at-risk condition aged ≥ 18 years, non-Indigenous adults aged ≥ 70 years and Aboriginal and Torres Strait Islander adults aged ≥ 25 years. The PBAC's recommendation for listing the existing NIP populations (individuals with an at-risk condition aged ≥ 18 years, non-Indigenous adults aged ≥ 70 years and Aboriginal and Torres Strait Islander adults aged ≥ 50 years) was based on, among other matters, its assessment that the cost-effectiveness of 20vPCV would be acceptable if it were cost-minimised against the nominated comparators, 13-valent pneumococcal conjugate vaccine (Prevenar 13®) and 15-valent pneumococcal conjugate vaccine (Vaxneuvance®). The PBAC acknowledged the disproportionately high burden of pneumococcal disease in the proposed expanded NIP population, Aboriginal and Torres Strait Islander adults aged 25 - 49 years, and recommended listing on the basis that 20vPCV, with or without one or two subsequent doses of 23vPPV, would be cost-effective at the cost per dose proposed in the sponsor's pre-PBAC response.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
POLATUZUMAB VEDOTIN Powder for I.V. infusion 30 mg Powder for I.V. infusion 140 mg Polivy® ROCHE PRODUCTS PTY LTD Category 2 submission (New PBS listing)	Diffuse large B-Cell lymphoma	To request a Section 100 (Efficient Funding of Chemotherapy Program) Authority Required (STREAMLINED) listing for use in combination with rituximab, cyclophosphamide, doxorubicin and prednisone, for the treatment of previously untreated diffuse large B-cell lymphoma.	Not Recommended	The PBAC did not recommend the listing of polatuzumab vedotin in combination with rituximab plus cyclophosphamide, doxorubicin and prednisone (Pola+R-CHP) for the treatment of diffuse large B-cell lymphoma (DLBCL) in previously untreated patients with an international prognostic index (IPI) score of 3-5. The PBAC considered that Pola+R-CHP did not provide a benefit compared to the comparator, rituximab plus cyclophosphamide, doxorubicin, vincristine and prednisone (R-CHOP), in terms of overall survival and overall response rate. In addition, the PBAC considered that the estimated incremental cost-effectiveness ratio was optimistic and the financial impact of listing polatuzumab vedotin was high. <u>Sponsor's Comment:</u> The sponsor had no comment.
RANIBIZUMAB Solution for ocular implant 39.5 mg in 0.395 mL Susvimo® ROCHE PRODUCTS PTY LTD Matters Outstanding (New PBS listing) To be considered at a future PBAC meeting	Neovascular (wet) age-related macular degeneration	To request 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.	Not Applicable	This item is to be considered at a future PBAC meeting.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
RISANKIZUMAB Solution concentrate for I.V. infusion 600 mg in 10 mL Injection 360 mg in 2.4 mL in pre-filled cartridge Skyrizi® AbbVie Pty Ltd Matters Outstanding (New PBS listing)	Crohn disease	To request General Schedule Authority Required (Written) listings for the treatment of severe Crohn disease and for complex refractory fistulising Crohn disease.	Recommended	The PBAC recommended the Section 100 (Highly Specialised Drugs Program) (intravenous administration) and General Schedule (subcutaneous administration) listings of risankizumab (RIS) for the treatment of severe Crohn disease, on a cost-minimisation basis with the least costly alternative biologic or targeted synthetic disease modifying anti-rheumatic drug (bDMARD/tsDMARD). The PBAC considered the claim of non-inferior comparative effectiveness and safety to the alternative bDMARDs/tsDMARDs was reasonable. The PBAC's recommendation was therefore, among other matters, based on its assessment that the cost of RIS should be no greater than the cost of the alternative therapies over two years. The PBAC recommended flow-on changes to other bDMARD/tsDMARD listings in severe Crohn disease to include RIS in the list of eligible treatments in a treatment cycle.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
RISEDRONIC ACID Tablet (enteric coated) containing risedronate sodium 35 mg Actonel® EC THERAMEX AUSTRALIA PTY LTD Category 2 submission (Change to PBS listing)	Osteoporosis	To request an expansion of the current risedronate listing to include patients with osteoporosis aged below 70 years of age.	Deferred	The PBAC deferred making a recommendation to amend the current age restriction on the PBS listing of risedronic acid (risedronate) for primary prevention of fracture in patients with a bone mineral density (BMD) T-score of -2.5 or less. The PBAC advised it was of a mind to support amending the current age restriction of risedronate from patients aged 70 years and older, to patients aged 60 years based on the fracture risk in these patients. However, the PBAC deferred consideration pending a review of the MBS implications, to ensure that the bone densitometry MBS items could be aligned with the PBAC recommendations. The PBAC noted the cost-effectiveness of risedronate for the expanded population needs to be assessed considering the absolute fracture risk in these patients, the cost of alternative therapies and the impact of BMD screening. <u>Sponsor's Comment:</u> Theramex look forward to working with the PBAC to bring anti-resorptive medicine to a broader group of patients.
RUXOLITINIB Tablet 5 mg Tablet 10 mg Jakavi® Novartis Pharmaceuticals Australia Pty Limited Matters Arising (Change to PBS listing)	Graft versus host disease	To request a General Schedule Authority Required (STREAMLINED) listing for the treatment of patients aged 12 years and older with moderate to severe chronic graft versus host disease (cGVHD) who are refractory to, dependent on or intolerant of corticosteroids.	Recommended	The PBAC recommended the listing of ruxolitinib for the treatment of patients with moderate to severe chronic graft versus host disease who are refractory to, dependent on, or intolerant of corticosteroids. The PBAC was satisfied that ruxolitinib provides, for some patients, a significant improvement in efficacy, including an improvement in overall response rate, compared with best available therapy. The PBAC considered that ruxolitinib would be acceptably cost-effective at the price proposed. Further, the PBAC considered that the changes to the financial estimates and risk sharing arrangements were reasonable.

DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION	DRUG TYPE AND USE	LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION	PBAC OUTCOME	
SELINEXOR Tablet 20 mg Xpovio® ANTENGENE (AUS) PTY. LTD. Standard re-entry submission (New PBS listing)	Multiple myeloma	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 relapsed and/or refractory multiple myeloma (RRMM).	Recommended	The PBAC recommended the listing of selinexor, for use in combination with bortezomib and dexamethasone (SBd), for the treatment of patients with RRMM who have received at least one prior therapy. The PBAC noted that multiple myeloma is heterogeneous disease and that there is a clinical need for multiple treatment options, including different drug classes. The PBAC considered that there should be a single listing for selinexor which allows use in patients as a second or later line treatment either with or without bortezomib to enable clinicians to utilise the appropriate regimen for their patient (either SBd or selinexor in combination with dexamethasone) based on clinical judgement. The PBAC considered that the claim of non-inferior effectiveness versus carfilzomib with dexamethasone (Cd) was uncertain due to the transitivity issues across the trials and the wide confidence intervals for the indirect estimates. However, the PBAC accepted the effectiveness claim on the basis that further data are unlikely to be available to increase the certainty for the non-inferiority claim and that SBd will be used in a relatively small niche population. The PBAC noted that the revised cost-minimisation analysis (CMA) was sufficiently conservative to address the uncertainty regarding the non-inferiority claim versus Cd and considered that SBd could be considered cost-effective if the selinexor price was determined on the basis of the CMA of SBd vs Cd presented in the resubmission, after inclusion of the confidential effective price for carfilzomib and an adjusted weighting for the use of carfilzomib once weekly and twice-weekly regimens.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
SELUMETINIB Capsule 10 mg Capsule 25 mg Koselugo® Alexion Pharmaceuticals Australasia Pty Ltd Category 1 submission (New PBS listing)	Neurofibromatosis type 1 (NF1)	To request both a Section 100 (Highly Specialised Drugs Program) and Section 100 (Highly Specialised Drugs Program – Community Access) Authority Required listing for the treatment of symptomatic, inoperable plexiform neurofibroma(s) in paediatric patients with NF1.	Not Recommended	The PBAC did not recommend selumetinib for the treatment of symptomatic, inoperable plexiform neurofibroma(s) (PN) in paediatric patients with neurofibromatosis (NF1). Although the PBAC considered that selumetinib provided a high clinical benefit for patients with PN associated with NF1 compared to supportive care, the PBAC noted that there were significant issues with the proposed restriction, the economic model and the utilisation estimates. The PBAC also noted that there were quality use of medicine issues relating to the size of the capsule and the proposed use in young children (who were the most likely to benefit from treatment). The PBAC nominated the Facilitated Resolution pathway for this item given the high added therapeutic value and significant outstanding issues. <u>Sponsor's Comment:</u> Alexion welcomes the PBAC's acknowledgment that selumetinib provides a high clinical benefit for paediatric patients with rare plexiform neurofibroma(s) (PN) with neurofibromatosis type 1 (NF1). We look forward to continuing to work with the PBAC and the Department to secure access to selumetinib for children with this rare condition, where there are no alternative therapies available

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 2022 PBAC MEETING

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SOTORASIB Tablet 120 mg Lumakras® AMGEN AUSTRALIA PTY LIMITED Standard re-entry submission (New PBS listing) WITHDRAWN	Non-small cell lung cancer	Resubmission to request a General Schedule Authority Required listing for the treatment of Kirsten rat sarcoma (KRAS) G12C variant non-squamous or not otherwise specified stage IIIB (locally advanced) or stage IV (metastatic) non-small cell lung cancer in patients who have progressed on prior therapy.	Not Applicable	This item was withdrawn.
UPADACITINIB Tablet 15 mg Tablet 30 mg Tablet 45 mg Rinvoq® AbbVie Pty Ltd Matters Outstanding (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), in patients who are contraindicated to, or whose disease has not adequately responded to conventional therapies.	Recommended	The PBAC recommended the General Schedule listings of upadacitinib (UPA) for the treatment of MSUC, on a cost-minimisation basis with the least costly alternative biologic or targeted synthetic disease modifying anti-rheumatic drug (bDMARD/tsDMARD) (excluding adalimumab (ADA)). The PBAC considered the claim of non-inferior comparative effectiveness to the alternative bDMARDs/tsDMARDs and superior comparative effectiveness to ADA were reasonable. The PBAC's recommendation was therefore, among other matters, based on its assessment that the cost of UPA should be no greater than the cost of the alternative therapies (excluding ADA) over two years. The PBAC recommended flow-on changes to other bDMARD/tsDMARD listings in MSUC to include UPA in the list of eligible treatments in a treatment cycle.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 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	
UPADACITINIB Tablet 15 mg Rinvoq® ABBVIE PTY LTD Category 2 submission (Change to PBS listing)	Non-radiographic axial spondyloarthritis	To request a General Schedule Authority Required (Written) listing for the treatment of non-radiographic axial spondyloarthritis (nr-axSpA).	Deferred	The PBAC deferred making a recommendation on the submission seeking to list upadacitinib (UPA) for nr-axSpA, as the TGA Delegate's Overview was not available at time of PBAC consideration. However, the PBAC was of a mind to recommend the listing of UPA on a cost-minimisation basis with the least costly alternative biological or targeted synthetic disease modifying anti-rheumatic drug for nr-axSpA because UPA is likely to be of overall non-inferior comparative effectiveness and safety to the alternative therapies. <u>Sponsor's Comment:</u> The sponsor had no comment.
VOSORITIDE Powder for injection 0.4 mg with diluent Powder for injection 0.56 mg with diluent Powder for injection 1.2 mg with diluent Voxzogo® BioMarin Pharmaceutical Australia Pty Ltd Matters Outstanding (New PBS listing)	Achondroplasia	To request a General Schedule Authority Required listing for the treatment of patients with achondroplasia whose epiphyses are not closed.	Recommended	The PBAC recommended the listing of vosoritide for the treatment of patients with achondroplasia whose epiphyses are not closed. The PBAC was satisfied that vosoritide provides, for some patients, a significant improvement in efficacy over best supportive care. The PBAC reaffirmed that there are no treatments on the PBS available specifically for this condition, and it considered that the addition of vosoritide offered high added therapeutic value. The PBAC's recommendation for listing was based on, among other matters, its assessment, that the cost-effectiveness of vosoritide would be acceptable at the price proposed in the September 2022 resubmission, if additional measures were implemented, including a review of trial evidence available at three years post the date of listing, and a risk sharing arrangement.

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 2022 PBAC MEETING

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DURVALUMAB Solution concentrate for I.V. infusion 120 mg in 2.4 mL; Solution concentrate for I.V. infusion 500 mg in 10 mL Imfinzi® AstraZeneca Pty Ltd	Small cell lung cancer	Review of positive PBAC recommendations not accepted by applicants	The PBAC advised that the November 2020 recommendation be extended for 6 months (if listing process has not commenced by the end of June 2023 the sponsor will be asked to justify the recommendation again).
ENOXAPARIN 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 syringe Injection containing enoxaparin sodium 150 mg (15,000 I.U. anti-Xa) in 1 mL syringe 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 syringe Enoxapro® Apotex Pty Ltd	Prevention of deep vein thrombosis; Haemodialysis	Review of positive PBAC recommendations not accepted by applicants	The PBAC advised that the July 2020 recommendation be extended for 12 months (if listing process has not commenced by the end of October 2023 the sponsor will be asked to justify the recommendation again).

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 2022 PBAC MEETING

DRUG NAME, FORM(S), STRENGTH(S) AND SPONSOR	DRUG TYPE AND USE	LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION	PBAC OUTCOME
FOSNETUPITANT (AS CHLORIDE HYDROCHLORIDE) / PALONOSETRON (AS HYDROCHLORIDE) Powder for Injection containing fosnetupitant 235 mg and palonosetron 250 mg Akynzeo IV® Mundipharma Pty Limited	Nausea and vomiting	Review of positive PBAC recommendations not accepted by applicants	The PBAC advised that the July 2020 recommendation could be revoked, noting that another form of the product would be considered at the March 2023 PBAC meeting.
FULVESTRANT Injection 250 mg in 5 mL pre-filled syringe Faslodex® AstraZeneca Pty Ltd	Breast cancer	Review of positive PBAC recommendations not accepted by applicants	The PBAC advised that the July 2020 recommendation could be revoked, noting that other brands of the product were available on the PBS.
IBRUTINIB Tablet 140 mg Tablet 280 mg Tablet 420 mg Tablet 560 mg Imbruvica® Janssen-Cilag Pty Ltd	Chronic lymphocytic leukaemia; Small lymphocytic lymphoma; Mantle cell lymphoma	Review of positive PBAC recommendations not accepted by applicants	The PBAC advised that the November 2020 recommendation be extended for 12 months (if listing process has not commenced by the end of October 2023 the sponsor will be asked to justify the recommendation again).

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 2022 PBAC MEETING

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PANCREATIC EXTRACT Capsule (containing enteric coated minimicrospheres) providing not less than 20,000 BP units of lipase activity Creon® Mylan Health Pty Ltd	Pancreatic exocrine insufficiency	Review of positive PBAC recommendations not accepted by applicants	To be considered at a future PBAC meeting

Version 3
Amendment

1. GLYCOMACROPEPTIDE FORMULA WITH LONG CHAIN POLYUNSATURATED FATTY ACIDS AND DOCOSAHEXAENOIC ACID AND LOW IN PHENYLALANINE (PKU Sphere Liquid) – Drug name amended

Previous Amendments

1. DAROLUTAMIDE (Nubeqa®) – New outcome
2. LENACAPAVIR (Sunlenca®) – New outcome

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) MEETING OUTCOMES
NOVEMBER 2022 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 HATV for the proposed population. Applicants who accept this pathway are eligible for PBAC consideration at the immediate next meeting.
Early resolution pathway	For medicines or vaccines deemed by the PBAC to represent High Added Therapeutic Value (HATV) AND where the PBAC considers that the remaining issues could be easily resolved, including when: • new clinical study data requiring evaluation is not considered necessary by the PBAC to support new clinical claims to be made in the resubmission; and • a revised model structure or input variable changes (beyond those specified by the PBAC) are not necessary to support any new economic claims, or to estimate the utilisation and financial impacts to be made in the resubmission. Applicants who accept this pathway are eligible for PBAC consideration out-of-session (before the main meeting), unless the department, in consultation with the PBAC Chair, identifies an unexpected issue such that the resubmission needs consideration at the next main PBAC meeting.
Facilitated resolution pathway	A Facilitated Resolution Pathway may be nominated by the PBAC where the PBAC considers the issues for resolution could be explored through a workshop AND where the medicine or vaccine meets the HATV criteria. Applicants who accept this pathway are eligible for a solution-focussed workshop with one or more members of the PBAC. The workshop agenda will be based on the issues for resolution outlined in the PBAC Minutes. This can be further clarified during the post-PBAC meeting with the Chair.