

JULY 2020 PBAC OUTCOMES – 1ST TIME DECISIONS NOT TO RECOMMEND

DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION	DRUG TYPE AND USE	LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION	PBAC OUTCOME
<p>ACALABRUTINIB</p> <p>Capsule 100 mg</p> <p>Calquence®</p> <p>AstraZeneca Pty Ltd</p> <p>New listing (Major Submission)</p>	<p>Chronic lymphocytic leukaemia (CLL) or small lymphocytic lymphoma (SLL)</p>	<p>To request an Authority Required listing for the treatment of patients (either as monotherapy or in combination with obinutuzumab) with previously untreated CLL or SLL considered unsuitable for treatment with a purine analogue. A second request was for use only in the subgroup of patients with a 17p deletion.</p> <p align="center">Sponsor's comment:</p>	<p>The PBAC did not recommend the listing of acalabrutinib, for use as monotherapy or in combination with obinutuzumab, for the first-line treatment of patients with CLL or SLL who are considered unsuitable for treatment with a purine analogue. The PBAC considered that the incremental cost-effectiveness ratio was unacceptably high and uncertain at the proposed price.</p> <p>The PBAC did not recommend the listing of acalabrutinib, for use as monotherapy or in combination with obinutuzumab, for the first-line treatment of patients with CLL or SLL who harbour a 17p deletion as non-inferiority versus ibrutinib was not adequately demonstrated.</p> <p>The sponsor had no comment.</p>
<p>CAPLACIZUMAB</p> <p>Injection set containing 1 vial powder for injection 10 mg and 1 pre-filled syringe solvent 1 mL</p> <p>Cablivi®</p> <p>Sanofi-Aventis Australia Pty Ltd</p> <p>New listing (Major Submission)</p>	<p>Acquired thrombotic thrombocytopenic purpura (aTTP)</p>	<p>To request a Section 100 (Highly Specialised Drugs Program - Public and Private Hospitals) Authority Required listing for the treatment of adult patients with aTTP.</p> <p align="center">Sponsor's comment:</p>	<p>The PBAC did not recommend the listing of caplacizumab for the treatment of aTTP. The PBAC considered the clinical place of therapy was uncertain. The PBAC had low confidence in the trial data given trial design, size, and conduct issues. The clinical benefit in the acute setting was considered modest at best and the long-term clinical benefits of treatment remained unclear, given the clinical trial results did not demonstrate a significant improvement in end organ damage and mortality. The PBAC also considered the cost-effectiveness of caplacizumab to be high and uncertain due to the significant structural issues in the economic model, which favoured caplacizumab.</p> <p>While Sanofi is disappointed with the PBAC's decision not to recommend Cablivi®, we look forward to continuing to work with the Committee and the Department of Health to make Cablivi® available to Australians with this rare and life-threatening disease.</p>

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<p>DAROLUTAMIDE</p> <p>Tablet 300 mg</p> <p>Nubeqa®</p> <p>Bayer Australia Pty Ltd</p> <p>New listing (Major Submission)</p>	<p>Prostate cancer</p>	<p>To request an Authority Required listing for the treatment of non-metastatic castration-resistant prostate cancer (m0CRPC).</p>	<p>The PBAC did not recommend the listing of darolutamide for the treatment of patients with m0CRPC who are at high risk of distant metastases. While the PBAC considered that darolutamide provides a substantial benefit for some patients compared with standard of care in terms of delaying disease progression, it considered that the magnitude of the overall survival benefit was modest, and uncertain given the immaturity of the data. The PBAC considered that the modelled survival benefit was likely overestimated, the incremental cost-effectiveness ratio was high and underestimated, and that a substantial price reduction would be required for darolutamide to be considered cost-effective. Further, the PBAC considered that the estimated financial impact of darolutamide to the PBS was uncertain.</p>
		<p align="center">Sponsor's comment:</p>	<p>Bayer will continue to work with the PBAC and the Department of Health on the listing of darolutamide.</p>
<p>MOGAMULIZUMAB</p> <p>Solution concentrate for I.V. infusion 20 mg in 5 mL</p> <p>Poteligeo®</p> <p>Kyowa Kirin Australia Pty Ltd</p> <p>New listing (Major Submission)</p>	<p>Cutaneous T-Cell Lymphoma (CTCL)</p>	<p>To request a Section 100 (Efficient Funding of Chemotherapy) Authority Required (Written) listing for patients with relapsed or refractory CTCL who have been previously treated with at least one prior systemic therapy.</p>	<p>The PBAC did not recommend the listing of mogamulizumab for the treatment of patients with relapsed or refractory CTCL following at least one prior systemic treatment for this condition. The PBAC considered that the extent of benefit for mogamulizumab in terms of progression free survival and overall survival was uncertain. In addition, the PBAC considered the incremental cost-effectiveness ratio was unacceptably high and uncertain at the proposed price, and the estimated financial impact was uncertain.</p>
		<p align="center">Sponsor's comment:</p>	<p>While disappointed with the outcome, Kyowa Kirin remains committed to working with the PBAC to provide access to mogamulizumab for Australians living with relapsed or refractory CTCL.</p>

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<p>RAVULIZUMAB</p> <p>Solution concentrate for I.V. infusion 300 mg in 30 mL</p> <p>Ultomiris®</p> <p>Alexion Pharmaceuticals Australasia Pty Ltd</p> <p>New listing (Major Submission)</p>	<p>Paroxysmal nocturnal haemoglobinuria (PNH)</p>	<p>To request a Section 100 (Highly Specialised Drugs Program - Public and Private Hospitals) Authority Required listing for the treatment of adult patients with PNH.</p>	<p>The PBAC did not recommend the listing of ravulizumab for the treatment of patients with PNH. The PBAC considered the clinical evidence presented in the submission demonstrated that ravulizumab is likely to be non-inferior compared to eculizumab in the short-term. However, the PBAC considered that due to the lack of long-term follow-up data available, it was uncertain whether a patient's lifespan would be substantially extended as a direct consequence of the use of ravulizumab. The PBAC also considered the applicant's economic model did not provide a valid means of estimating the cost-effectiveness of ravulizumab for PBS listing and was therefore not informative for decision-making.</p>
		<p>Sponsor's comment:</p>	<p>Alexion welcomes the PBAC's acknowledgement that ravulizumab is non-inferior to eculizumab, however is disappointed with the PBAC's decision not to recommend. Alexion is committed to working with the PBAC and the Department of Health to pursue access for adult patients with PNH who are calling for this new bi-monthly treatment option which will reduce the burden of fortnightly infusions.</p>
<p>TAFAMIDIS</p> <p>Capsule 61 mg</p> <p>Vyndamax®</p> <p>Pfizer Australia Pty Ltd</p> <p>New listing (Major Submission)</p>	<p>Transthyretin amyloid cardiomyopathy (ATTR-CM)</p>	<p>To request an Authority Required listing for the treatment of ATTR-CM.</p>	<p>The PBAC did not recommend the listing of tafamidis for the treatment of patients with ATTR-CM. The PBAC accepted that tafamidis was clinically superior to current standard management, and considered there was a high unmet clinical need for effective therapies for ATTR-CM.</p>
		<p>Sponsor's comment:</p>	<p>However, the PBAC considered that the incremental cost-effectiveness ratio had not been reliably estimated and was unacceptably high at the price proposed in the submission. The PBAC considered that the financial impact was highly likely to have been significantly underestimated by the submission given the uncertain prevalence of this condition and the potential for diagnosis to be increased with the new diagnostic algorithm proposed.</p>
		<p>Sponsor's comment:</p>	<p>Pfizer welcomes the PBAC's acknowledgement of the high unmet clinical need for effective therapies for ATTR-CM and acceptance of the superiority of tafamidis over standard management. Pfizer will continue to work collaboratively with the PBAC to deliver access to tafamidis for patients with this debilitating and life-threatening condition.</p>