

**NOVEMBER 2020 PBAC OUTCOMES - DEFERRALS**

<b>DRUG NAME, FORM(S), STRENGTH(S), SPONSOR, TYPE OF SUBMISSION</b>	<b>DRUG TYPE AND USE</b>	<b>LISTING REQUESTED BY SPONSOR / PURPOSE OF SUBMISSION</b>	<b>PBAC OUTCOME</b>
<p>ADALIMUMAB</p> <p>Injection 40 mg pre-filled syringe Injection 40 mg auto-injector</p> <p>Hadlima®</p> <p>Merck Sharp &amp; Dohme (Australia) Pty Ltd</p> <p>Change to listing (Minor Submission)</p>	<p>Severe Crohn disease; complex refractory fistulising Crohn disease; moderate to severe ulcerative colitis; severe active juvenile idiopathic arthritis; adult patients with a history of juvenile idiopathic arthritis; severe psoriatic arthritis; ankylosing spondylitis; severe chronic plaque psoriasis; severe active rheumatoid arthritis</p>	<p>To request an Authority Required (STREAMLINED) listing for the biosimilar in the continuing treatment phase; to request an Authority Required (telephone) for the biosimilar in the initial treatment phase; and to request that use of the biosimilar not count as treatment failure.</p>	<p>The PBAC deferred its consideration of the requested biosimilar uptake drivers for adalimumab (Hadlima®) on the basis that these matters had potentially broader biosimilar policy implications and required further discussions between the Department and relevant stakeholders to ensure appropriate consideration.</p> <p>The PBAC considered that further work should take place out of session and return to the PBAC at a later date.</p>
		<p align="center">Sponsor's comment:</p>	<p>MSD is disappointed the PBAC has once again deferred consideration of additional biosimilar uptake drivers to support patient and clinician use of biosimilars in line with government policy. We acknowledge the PBAC has reiterated its request for additional consultation between the Department and relevant stakeholders, which should take place as soon as possible.</p> <p>We remain ready to assist in these discussions to progress MSD's proposal and its implementation. This will ensure patients and the health system benefit from the savings generated by the uptake of biosimilars in a timely manner.</p>
<p>ADRENALINE</p> <p>I.M. injection 150 micrograms in 0.3 mL single dose syringe auto-injector I.M. injection 300 micrograms in 0.3 mL single dose syringe auto-injector I.M. injection 500 micrograms in 0.3 mL single dose syringe auto-injector</p> <p>Anapen®</p> <p>Allergy Concepts Pty Ltd</p>	<p>Acute allergic reaction with anaphylaxis</p>	<p>To request the Authority Required listing of an alternative brand of adrenaline auto-injector under the same conditions as other brands of adrenaline currently listed on the PBS and to request the Authority Required listing of a new strength of adrenaline auto-injector.</p>	<p>The PBAC deferred making a recommendation to list adrenaline auto-injectors, Anapen Junior®, Anapen 300® and Anapen 500®, as the TGA Delegate's Overview was not available at the time of consideration. However, the PBAC was of a mind to recommend the General Schedule, Authority Required listing of adrenaline auto-injectors Anapen 300, Anapen 500 and Anapen Junior for treatment of acute allergic reaction with anaphylaxis.</p> <p>The PBAC noted that consideration of medicines in parallel with the TGA is usually restricted to major submissions, however in light of the ongoing supply issues for PBS listed brands of adrenaline PFS auto-injectors, this submission was accepted prior to receiving the Delegate's Overview.</p>
<p>New listing (Minor Submission)</p>		<p align="center">Sponsor's comment</p>	<p>The sponsor had no comment.</p>

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<p>GLECAPREVIR + PIBRENTASVIR</p> <p>Tablet containing 100 mg glecaprevir with 40 mg pibrentasvir</p> <p>Maviret®</p> <p>AbbVie Pty Ltd</p> <p>Change to listing (Minor Submission)</p>	<p>Chronic hepatitis C infection</p>	<p>To request an amendment to the Section 100 (Highly Specialised Drugs Program) and General Schedule Authority Required listings to reduce the duration of treatment from 12 weeks to 8 weeks for treatment naïve patients with chronic hepatitis C with compensated cirrhosis.</p>	<p>The PBAC was of a mind to recommend the listing of an 8-week treatment option of glecaprevir with pibrentasvir (GLE/PIB) for the treatment of chronic hepatitis C (CHC) infection in patients who are treatment-naïve with compensated cirrhosis (TN/CC), however, deferred making a recommendation to amend the General Statement for Drugs for the Treatment of Hepatitis C (the General Statement) pending finalisation of the TGA registration.</p> <p>The PBAC noted there was residual uncertainty as to whether the 12-week regimen would remain part of the TGA registration and was of a mind to retain the listing of the 12-week regimen if it were retained for this population.</p>
		<p align="center">Sponsor's comment:</p>	<p>AbbVie welcomes the PBAC's minded recommendation for 8-week treatment duration for patients who are treatment-naïve with compensated cirrhosis (TN/CC), and acknowledges that the TGA has also recommended that the 12 week regimen may be considered for those patients at the discretion of the prescriber, and that the PBAC proposes that this would be included as a footnote in the General Statement for simplicity.</p>
<p>LAROTRECTINIB</p> <p>Capsule 25 mg Capsule 100 mg Oral solution 20 mg per mL, 100 mL</p> <p>Vittrakvi®</p> <p>Bayer Australia Ltd</p> <p>New listing (Major submission)</p>	<p>Solid tumours harbouring neurotrophic receptor tyrosine kinase (NTRK) gene fusions</p>	<p>To request a Section 100 (Highly Specialised Drugs Program) Authority Required (Written) listing for the treatment of locally advanced or metastatic solid tumours harbouring tropomyosin receptor kinase NTRK gene fusions.</p>	<p>The PBAC deferred its decision about whether to recommend the listing of larotrectinib for the treatment of patients with NTRK fusion tumours that are either unresectable locally advanced, metastatic, or locally advanced and would otherwise require disfiguring surgery or limb amputation to achieve a complete surgical resection. The PBAC acknowledged there is a high clinical need for effective treatments for patients with NTRK fusion tumours noting that NTRK fusions are found in a number of rare cancer types. The PBAC noted that the limited clinical data suggested the potential comparative treatment benefit of larotrectinib would mostly be in paediatric patient populations and adult patients with tumours harbouring NTRK fusions at high frequency. The PBAC was of a mind to not recommend the listing of larotrectinib on the basis that the cost-effectiveness ratio was unacceptably high and uncertain at the price proposed. The PBAC requested the Department approach the sponsor to seek a suitable price reduction for larotrectinib. The PBAC also advised it would await the Medical Services Advisory Committee (MSAC) advice on the funding of the codependent NTRK testing before making a final decision on PBS funding.</p>

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		Sponsor's comment:	Bayer continues to work collaboratively with the PBAC and the Department of Health, to achieve sustainable PBS listing conditions and to enable patients with an NTRK fusion cancer in Australia to receive access to larotrectinib at the earliest opportunity.
<p>OBETICHOLIC ACID</p> <p>Tablet 5 mg Tablet 10 mg</p> <p>Ocaliva®</p> <p>Chiesi Australia Pty Ltd</p> <p>New listing (Major Submission)</p>	Primary biliary cholangitis	<p>Resubmission to request an Authority Required (Written) listing for the treatment of primary biliary cholangitis (PBC).</p> <p>Sponsor's comment:</p>	<p>The PBAC deferred making a recommendation regarding the listing of obeticholic acid as a second-line treatment of PBC. Although the PBAC again acknowledged that there was a clinical need for effective PBC treatments, the PBAC considered that a further price reduction would be required to address the uncertainties relating to the magnitude of the clinical benefit, the incremental cost effectiveness ratio (ICER) and the estimated financial impact.</p> <p>Chiesi Australia welcomes the deferral and will work with the PBAC to ensure patient access to this effective medicine.</p>
<p>ONASEMNOGENE ABEPARVOVEC</p> <p>Solution for injection, customised based on patient weight</p> <p>Zolgensma®</p> <p>Novartis Pharmaceuticals Australia Pty Ltd</p> <p>New listing (Major Submission)</p>	Spinal muscular atrophy (SMA)	Submission to request an Authority Required (Written) listing for the treatment of paediatric patients less than 2 years of age with Type 1 SMA.	<p>The PBAC deferred making a decision on the request for PBS listing of onasemnogene abeparvovec (ONA) (Zolgensma®) in patients less than 2 years of age with confirmed SMA (based on genotype and phenotype) to allow:</p> <ol style="list-style-type: none"> <li>1. The Therapeutic Good Administration (TGA) to finalise key aspects of the indication for which the therapy will be registered including: patient age at time of treatment; number of copies of the survival motor neuron 2 (SMN2) gene; defined SMA type and symptomatic versus pre-symptomatic status. This information is essential for PBAC to provide advice on which patients should be eligible for PBS treatment and to finalise its assessment of the effectiveness and cost-effectiveness of treatment in eligible patients.</li> <li>2. Stakeholders to work together to develop an overall and holistic approach to the treatment pathway and clinical evidence for SMA that takes into account the new treatment options that have become available over the past five years as well as developments in newborn screening and pre-conception and early pregnancy testing.</li> </ol>

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			<p>The PBAC noted that it first considered a subsidy proposal for the SMA treatment, nusinersen, in 2017-2018. Prior to that time, there were no direct treatments for this condition. However, since that time, PBAC has considered six more SMA subsidy proposals, including the two considered at this meeting. Further subsidy proposals are expected in the near future.</p> <p>The new subsidy proposals coming to PBAC are both for different groups of SMA patients, including pre-symptomatic and older patients, and for new treatments. The new treatments expected to become available for SMA in Australia include include ONA and branaplam (both Novartis products) and risdiplam (Roche).</p> <p>Alongside these developments, NSW and the ACT have conducted a pilot new-born screening program for SMA, and the Medical Services Advisory Committee (MSAC) has recommended public funding for reproductive carrier testing to detect cystic fibrosis, SMA and fragile X syndrome pathogenic variants in women early in pregnancy or intending to become pregnant, and in their reproductive partners as needed (see MSAC application No. 1573 at <a href="http://www.msac.gov.au">www.msac.gov.au</a>).</p> <p>In light of these rapid developments in treatment and diagnosis, the PBAC considered it was in the interests of patients and families, prescribers and payers for a decision support analysis for SMA treatment that takes into account all the currently available clinical data and informs a broader “whole of disease” economic and financial analyses.</p> <p>The PBAC requested the Department convene a stakeholder meeting in the very near future including clinical experts, consumer representatives and relevant sponsors with the intention of progressing work on this decision support analysis.</p> <p>The PBAC also had a number of concerns with the current subsidy proposal for ONA. Most importantly, the PBAC did not accept the submission’s claim that ONA is superior in terms of effectiveness and safety as compared to nusinersen. Overall, having considered all the available evidence, PBAC concluded that, on balance ONA would likely deliver similar clinical outcomes to nusinersen in matched patients.</p>

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			<p>The PBAC recognised the high clinical need for effective treatments to treat Type I SMA and noted that ONA is a new in class therapy, which will expand the options available for patients and their families. The PBAC considered that in the absence of substantial new clinical data, which is unlikely to be forthcoming, a simple “frame of reference” cost-comparison with nusinersen may provide a way forward.</p>
<p>SAPROPTERIN</p> <p>Powder for oral solution 500 mg Tablet (soluble) 100 mg</p> <p>Kuvan®</p> <p>Biomarin Pharmaceutical Australia Pty Ltd</p> <p>Change to listing (Minor Submission)</p>	<p>Maternal phenylketonuria (MPKU)</p>	<p>Resubmission to request an Authority Required listing in combination with a phenylalanine (Phe)-restricted diet for the treatment of MPKU where a Phe-restricted diet does not adequately reduce blood Phe levels.</p>	<p>Novartis is committed to working with the PBAC to achieve a listing for Zolgensma.</p> <p>The PBAC deferred making a recommendation to list sapropterin in combination with a Phe-restricted diet for the treatment MPKU in order to consult further on the appropriate restriction, particularly: eligibility and processes for initial responsiveness testing; defining the circumstances of use post-partum; and the most equitable duration of therapy. As part of the deferral, the PBAC requested the sponsor provide further information regarding the feasibility of the proposed restriction for clinicians in practice.</p> <p>The PBAC noted there was a high clinical need in a small patient population, and acknowledged the input received from individuals, organisations and health professionals. Further, the PBAC noted the strong consumer feedback describing the very high clinical need for access to sapropterin for any adult with PKU. The PBAC would welcome a major resubmission for this broader population.</p>
		<p>Sponsor’s comment:</p>	<p>BioMarin looks forward to working with the PBAC to enable PBS access for this important patient group.</p>