The PBS Post Market Review team
PBAC Guidelines Review
Pharmaceutical Evaluation Branch
Department of Health, Canberra, ACT

PBSpostmarket@health.gov.au

14th September 2015

Dear Sir / Madam,

Re: Public Consultation on the Pharmaceutical Benefits Advisory Committee (PBAC) Guidelines Review
(see at: http://www.pbs.gov.au/info/reviews/pbac-guidelines-review and
http://www.pbs.gov.au/info/reviews/pbac-guidelines-review-public-consultation)

For more than 230 years, Takeda has been committed to serving the community through healthcare solutions from prevention to care to cure. Takeda has a broad portfolio of more than 700 products spanning a variety of therapeutic areas, including cardiovascular/metabolic, immunology/respiratory, central nervous system, general medicine and oncology. Takeda also aspires to deliver medicines that address unmet medical needs, such as our current highly specialised medicines in the therapeutic areas of haematological malignancies and Inflammatory Bowel Disease, and has a large research and development (R&D) program investigating a number of new medicines.

As an interested stakeholder, Takeda thanks the Department of Health (DoH) for enabling the opportunity to provide input towards the PBAC Guidelines Review (hereinafter the Review). As one of the member companies of Medicines Australia (MA)[1], Takeda fully supports the content and recommendations contained in the MA submissions to the Review, including the submission lodged 14 September 2015 and the MA summary paper of issues tabled at the initial Guidelines Review Steering Committee (GRSC) meeting in July 2015 (included as Appendix 1 in MA's submission).

Although the PBAC Guidelines have undergone periodic revisions, Takeda believes these have not been of sufficient breadth, nor has there been input from all the relevant stakeholders. In consequence, the current Guidelines do not enable the use of the technological / methodological approaches used by other health technology assessment (HTA) jurisdictions for evidence assessment / economic evaluation, nor have they incorporated the perspectives of all the stakeholders. Not only does this mean we believe

[1] Medicines Australia represents the research-based pharmaceutical industry in Australia, which brings new medicines, vaccines and health services to the Australian market.

Australia has slipped behind in terms of HTA, but it introduces additional challenges for an affiliate such as ours because the evidence packages / economic evaluations developed by our Global organisations are done to meet the requirements of other HTA bodies in the bigger countries such as the United Kingdom (UK), Canada and in the European / Nordic countries. Therefore, Australia's inability to directly implement such Global deliverables imposes an additional burden on local affiliates, occasioning extra cost and often results in a delay being able to submit to the PBAC.

Takeda believes that whilst the overall PBS process has served Australia well for many years, it is time to update many aspects of the process and the PBAC Guidelines and that these must be addressed soon if the Australian public is to receive innovative drugs in a timely manner.

Takeda notes that Items 1 and 2[2] in the Review do not explicitly capture the processes associated with a submission to the PBAC, including during the pre-lodgement phase (the Pre-PBAC processes) and those activities that happen after the PBAC's meeting, regardless of the latter's decision (the Post-PBAC processes). A proper understanding of all the processes relating to a PBAC submission is an integral part of the sponsor's role in partnering with DoH to enable the listing of medicines on the PBS. The current version of the PBAC Guidelines does outline some parts of the Pre-PBS listing process, but there are significant gaps and the Post-PBAC processes are either not captured at all, or are not transparent. If sponsors do not have clarity for what is required, by whom, and when; a delay is likely in the listing of a medicine following a positive recommendation by the PBAC. In addition, recent changes to PBAC processes (such as full electronic lodgement of a submission) need to be captured. Takeda appreciates the Government's wish to reduce red tape (as noted in the announcements of the Post-market Review of Authority Required PBS Listings[3] and the Review of TGA regulations[4]) and also the PBAC's stated desire for brevity and conciseness in the Guidelines[5]. However, a comprehensive 'processes' document (which should be a 'living' entity updated on a regular basis by the DoH) is not in conflict with the wish for a reduction in red tape and desire for brevity. Rather, the availability of such a document may actually lead to less need for the current interaction which occurs between the sponsors and the various sections of the DoH on 'administrative matters', thereby freeing up the DoH staff for more appropriate tasks. In alignment with the broader industry, Takeda believes a review / update of the PBAC Guidelines without an accompanying review and revision of the associated processes would be a missed

2 The following Items will be included in the Guidelines Review:

1. Review of the Guidelines content identifying the methods and current research. Identify significant new developments for methods in relevant sections of parts II and III of the current Guidelines (i.e. since 2008). In undertaking this service the Contractor will consider the relevance to PBAC practice of existing guidance documents on relevant methodologies contained within guidelines published by comparable international health technology assessment agencies, regulators and internationally recognised authorities in the assessment of evidence.
2. Preparation of a technical paper for discussion on each issue for the revision, including identifying any issues of scientific debate and consideration of Australian and International best practice. Recommendations on editorial and obsolete matters for deletion. Preparation of proposals for how to address each technical issue for the revision of the Guidelines undertaken by the Contractor for consultation with the Guidelines Review Steering Committee. The current version 4.4 of the Guidelines is a long document and the PBAC is seeking to develop a more concise, clear, focused and up-to-date methods Guidance to replace Parts II and III of version 4.4.

3 http://www.pbs.gov.au/info/reviews/authority-required-listings
4 http://www.pharmacynews.com.au/news/latest-news/government-announces-review-of-tga-regulations
5 http://www.pbs.gov.au/info/reviews/pbac-guidelines-review-public-consultation

opportunity and therefore encourages the DoH and the GRSC to consider broadening the current Review to undertake this assessment.

Based on feedback from Takeda colleagues in other affiliates in which HTA is also used as a decision-making tool for pharmaceutical reimbursement, Takeda believes the following are highly relevant to the current Review and we therefore ask the GRSC and DoH to consider these points within the Review.

- Enable the creation of a supporting unit for the PBAC (within the DoH) similar to the Decision Support Unit (DSU) implemented by the National Institute for Health and Care Excellence (NICE) in the UK (http://www.nicedsu.org.uk/). The DSU is commissioned by NICE to provide a research and training resource to support the Institute's Technology Appraisal Programme. The DSU is a collaboration between the Universities of Sheffield, York and Leicester. It also has members at the University of Bristol, London School of Hygiene and Tropical Medicine and Brunel University. It provides a variety of services which support the formal NICE evaluation process. The sections under the DSU 'umbrella' include Methods Development, Appraisal Specific Projects and Technical Support Documents. The public availability of this information (which is an academically rigorous synthesis of the agreed position for each topic) provides clarity and certainty to all the persons / entities involved in developing and evaluating a reimbursement application. The DSU also provides training on behalf of NICE, with this training being available to the NICE technical team and appraisal committee members and also to those submitting evidence, including industry, consultancies and academic groups. The training covers general issues around technology assessment and appraisals, the application of specific methods in areas such as evidence synthesis and the use of software for decision modelling.
- Enable the creation of a clinical panel which can provide expertise to the PBAC as required, similar to the remit of the Australian Technical Advisory Group on Immunisation (ATAGI)[6]. This clinical panel could be used to gain advice from clinical experts throughout Australia, both early on in the submission process (perhaps including involvement in the pre-PBAC submission meetings which are requested by sponsor companies) and on an on-going basis during the evaluation period when clinical points requiring clarification arise. This would be particularly helpful for therapeutic areas which are not represented by the members of the PBAC themselves. The Canadian HTA system (CADTH) employs clinical panels to provide advice[7]. The Scottish Medicines Consortium (the HTA body responsible for the funding of medicines in Scotland[8]) has initiated a process known as Patient and Clinician Engagement (PACE), whereby patient groups and clinicians can have a stronger voice in SMC decision making, especially for medicines used to treat cancer and very rare conditions. From May 2014 pharmaceutical companies have been able to request that SMC convenes a PACE group to review their medicine following the issue of the draft report from the formal SMC process.
- Enable the comprehensive inclusion of the consumer / societal viewpoint in a PBAC submission. As well as the perspectives of the consumer / carer / society being an important component of Section F of the current PBAC submission framework ("*Options to present additional relevant information*"), the economic evaluation should be able to incorporate these perspectives via the use of indirect costs and patient preference methodologies. This is particularly relevant in the context of a decision-making process which uses the cost per quality adjusted life year (cost / QALY) as a base metric because, in the majority of disease areas, the utility weights which drive

[6] https://www.ausgovboards.gov.au/boards/australian-technical-advisory-group-immunisation-atagi
[7] https://www.cadth.ca/
[8] https://www.scottishmedicines.org.uk/About_SMC/What_we_do/index

the cost / QALY reflect the various stages in the disease state itself and do not incorporate the patient's preference. For example, whilst the EORTC QLQ-C30 instrument is used to assesses the quality of life of cancer patients, it does not capture the 'utility' afforded to a patient by a new mode of administration such as being able to take a tablet at home rather than have to go to the hospital to receive an infusion. Whilst the cost offset of not having to go to hospital can be incorporated into the economic evaluation, the other, highly patient-relevant factors (such as the time saved by not having to travel to the hospital – time that may instead be productively spent at work/ the greater convenience / the lack of impact on the carer) cannot.

In conclusion, Takeda believes the Review offers the ideal opportunity for Australia to regain its status as a 'best-in-class' HTA country. This is particularly important if Australian patients are to receive timely access to new medicines in the years to come. Of note is the involvement of all stakeholders, which is commendable. Via such broad involvement, it is to be hoped the Review will identify the 'best-in-class' methods and innovative approaches taken in other countries, methods and approaches which can be implemented in Australia in the near term.

As noted in the MA submission, timely access to medicines is a shared goal of the pharmaceutical industry, the government, the PBAC, the clinical community and patients. Achieving this goal requires a predictable and transparent PBAC process which has as its core the use of sound methodological approaches to evidence assessment / economic evaluation and the 'best practices' used by other HTA countries to inform their decision-making. Takeda welcomes the commencement of the review and thanks the GRSC and the DoH for the opportunity to comment on the Review.

Yours sincerely,

James Jones,
Managing Director, Takeda Pharmaceuticals Australia