House of Representatives Standing Committee Inquiry into Access to Medicines
Pharmaceutical Benefits Advisory Committee Public Hearing
Summary of Issues

1. Background

1.1. The PBAC reminds the Inquiry that while the PBAC is an independent statutory advisory committee reporting to the Minister for Health, it operates and makes decisions within framework set by its legislative decision parameters and policies of the government of the day.

1.2. Those policy settings include the 2000 National Medicines Policy and notes the policy's first objective is 'timely access to the medicines that Australians need, at a cost individuals and the community can afford'.

1.3. The PBAC reviews each submission with the intent to recommend PBS listing where there is a clinical need as quickly as possible providing the submission demonstrates the medicine meets the requirements placed on the PBAC decisions by legislation.

1.4. The PBAC notes that while the PBAC is required by legislation to only make a recommendation for listing if it considers a medicine to be cost-effective, it does not have a mandated fixed threshold defining cost-effectiveness. The PBAC aims to be consistent and fair in its decision making across all medicines and clinical areas but also takes into account clinical need, rarity of medical conditions, and certainty of benefit and risks, meaning that different medicines may be recommended at different levels of evidence certainty and cost-effectiveness.

1.5. The PBAC notes that it is always open to suggestions for improvements in processes and seeks to respond to these where it can within the constraints of its legislated responsibilities, government policies and practical considerations such as commercial confidentiality.

1.6. The PBAC notes that the processes of the PBAC have continued to evolve and that there have been many changes in the past 4 years including in response to negotiations of government with industry bodies (strategic agreements), and initiatives of the PBAC. PBAC initiated changes include measures to increase patient engagement, patient hearings, increase transparency of information that informed PBAC decisions and implementing a process for review of PBAC recommendations which have not resulted in a PBS listing of a medicine.

1.7. The PBAC notes that the current evaluation process for submissions provides feedback to the sponsor at every step about where more information or clarity is needed, or other potential improvements to assist PBAC decision making. The sponsor is able to respond to that feedback prior to consideration at the

principle sub-committees (the Drug Utilisation Sub-Committee and the Economic Sub-Committee) and before the main PBAC meeting. Following the PBAC meeting, sponsors with submissions that did not receive a positive recommendation, are able to meet with the PBAC chair to discuss the reasons for the outcome and how these maybe addressed.

1.8. The PBAC notes recent changes to the processes for resubmission pathways and the post-PBAC listing processes which were negotiated by Government with industry. One of the new resubmission pathways provides for a facilitated resubmission for medicines deemed as having high added therapeutic value. These new pathways only came into effect in 2021 and their impact on resubmission rates and recommendations is not yet clear.

1.9. The PBAC notes that the guidelines for submissions to the PBAC for consideration of listing of a medicine on the PBS are regularly reviewed with wide consultation, most recently in 2015. The PBAC expects that such regular review should continue but notes the guidelines will and should always reflect the relevant legal and policy settings.

1.10. The PBAC notes that it is also conscious of the need for some stability and predictability in processes for applicants because of the time and resourcing involved in preparing submissions and to allow the impacts of individual changes to be assessed in their entirety.

1.11. The PBAC notes that the PBAC process is largely submission driven, that companies decide when they submit a medicine to Australia for registration and reimbursement, and for which indications they seek registration and reimbursement (and these maybe different). These aspects of Sponsors' decisions influence the time from TGA registration to PBS listings.

1.12. The PBAC is very aware of the patient and clinician desire for early access to medicines that appear on early evidence to be promising. Such submissions are often at an earlier time point in a medicine’s clinical development, where the evidence is often short term, often using surrogate endpoints, with greater uncertainty and less robust data. More robust data may be several years away from the time the PBAC is asked to make decisions.

1.13. The PBAC assessment, unlike the regulatory assessment which is on a balance of greater efficacy than harm, requires quantification of the size of the effect relative to what is already available. This inherent uncertainty has to be balanced against the patient and clinician desire and need for effective agents and our legislated requirement to ensure cost-effectiveness. Inevitably, conflicts in these differing priorities occur and may result in the PBAC decision being viewed as being unfair and unreasonable when it is actually balancing significant uncertainties which may have both health and financial related consequences.

1.14. Currently there is an absence of a nationally co-ordinated approach to horizon scanning for new important medicines that should be considered for health technology assessment. Given the boundaries between hospital and ambulatory care are becoming increasingly blurred, there is an increased need with some medicines to consider the shared benefits and costs between state health systems and the Commonwealth PBS/MBS. The PBAC notes the agreement of the Australian and State and Territory governments to explore a nationally cohesive health technology assessment approach and recommends medicines horizon scanning be included in this approach.

2. Possible Areas for Change for Inquiry consideration.

The PBAC is not a policy-making committee. In the spirit of this Inquiry, the PBAC provides the following comments based on our observations and experience with various products and processes over the years while noting that while some are within the remit of the PBAC, others require government consideration and decision.

2.1. Conditional recommendations, managed access programs and early access.

2.1.1. The creation of the TGA provisional registration pathway has created a situation where medicines judged by TGA to be a potential significant improvement for a high clinical need may be registered with very limited evidence on effectiveness and safety and generally no evidence of comparative benefits and harms.

2.1.2. The PBAC has noted that such medicines when considered by PBAC for PBS listing frequently come with submissions that have an Incremental Cost-Effectiveness Ratio well above the usual accepted level of cost effectiveness, justified by their purported high added therapeutic value. The decision parameters are then a recognised unmet clinical need, a cost-effectiveness that is already well below that of other medicines and significantly greater uncertainty due to the limited and less robust data.

2.1.3. Current legislation covering PBAC decisions does not specifically refer to conditional recommendations equivalent to the TGA provisional registration.

2.1.4. Under such circumstances, where there is a high unmet need (in PBAC terms that there are no existing PBS listed medicines for the indication and/or there is potential high added therapeutic value), PBAC may be asked or wish to consider a managed access program which aims to provide access while further evidence development is underway or undertaken.

2.1.5. This evidence could include the future results of a more definitive trial currently underway, new trials to address specific issues of comparative effectiveness, collection of new data on the effects observed in patients treated in Australia and/or the impacts of the new treatment on clinical practice in Australian practice.

2.1.6. Managed access programs that have requirements for further evidence collection are different to the risk share arrangements that are largely used to address concerns and uncertainties about effectiveness in populations outside

those examined in clinical trials, the uptake and therefore number of patients and total costs.

2.1.7. The PBAC would be more comfortable in its decision making if there was a specific legislative basis for conditional recommendations and managed access programs. This would also enhance transparency to about how and when such conditions could be applied. It would make it clearer to clinicians and patients that that continued listing may depend on their participation in additional data collection.

2.1.8. The PBAC notes that a number of submissions to the Inquiry refer to specific early access programs for promising new medicines. Noting this is a policy decision for government, based on our observations in countries with similar health systems to ours, such a program should have a legislated framework which is binding on sponsors in relation to negotiated entry price, the period and requirements for establishing a cost-effective price as determined by the PBAC, and agreement to continuation of supply for existing patients for free in the event that the cost-effective price is not agreed between parties. Such legislated frameworks have been implemented in similar government-funded reimbursement systems. Legislated frameworks will enable requirements for data collection and patient participation to be reasonable, relevant and mandated for the PBAC purposes. Such a program would require resourcing for clinical and patient participation, as well as the oversight of access protocols.

2.1.9. The PBAC notes that such special access programs as practiced in other countries may have a limit on the level of total financial exposure for Government in any year as a mechanism for controlling some of the uncertainty.

2.1.10. The PBAC strongly believes an early access program should not be limited to a specific disease or condition although the eligibility criteria of a medicine for such a program should refer to high unmet need and disease severity/prognosis.

2.2. Transparency, patient and clinician engagement, and inputs to informing decisions.

2.2.1. The PBAC notes the newly introduced patient engagement processes and the need to ensure that further expansion of these initiatives is adequately resourced. This includes that the confidentiality arrangements for submission can be modified to ensure that patient groups are adequately and accurately briefed about submissions.

2.2.2. A relatively simple matter that requires industry agreement is to inform clinician and patient groups early in the submission process of the specific indications for which reimbursement is being sought. This includes the clinical claim, intended populations, and details on proposed prescribing and clinician access requirements that the sponsor is proposing to PBAC for consideration.

2.2.3. The PBAC has reviewed patient engagement processes in other comparable jurisdictions and noted that the UK NHS and Scottish Medicine Collaboration both have processes that allow patient group representatives or individuals to observe part of committee deliberations. The PBAC is interested in trialling an

equivalent process while preserving applicants' expectations around confidentiality and is currently examining these practicalities, including resource implications for implementation.

2.2.4. Within existing processes, in the past 4 years the PBAC has undertaken an increased number of clinician and patient group consultations for submissions. These range from full stakeholder meetings to more rapid clinician consultations to refine or clarify clinical aspects of submissions. The PBAC has noted the considerable efforts that patient groups, and their membership have gone to engage with these processes, and the value of many of these representations to decision making.

2.2.5. The PBAC wishes to also acknowledge the willingness of many clinical groups to provide advice to the committee. It specifically notes the form of the contributions from the Medical Oncology Group of Australia (MOGA). MOGA provides specific advice for every new submission for medicines for solid cancers and notably has implemented a process for assessing the basis of their advice that is consistent with international best practice.

2.2.6. The PBAC is interested in exploring the mechanisms that might provide greater flexibility in committee membership without increasing what is already a large committee. This might include cross membership with MSAC to facilitate sharing of expertise especially for consideration of co-dependent submissions. The PBAC Drug Utilisation and Economic sub-committees already use guest discussants as a means of supplementing expertise.

2.3. Medicines for Rare Diseases.

2.3.1. The PBAC notes that a number of submissions to the Inquiry raised issues of access to medicines for rare or rarer diseases. The PBAC notes that some of these concerns relate to access to medicines which are not registered for their indication in Australia and/or where there is limited evidence of effectiveness and safety. The PBAC does not recommend PBS listing for specific indications for a medicine where that indication for the medicine is not registered by the TGA.

2.3.2. The PBAC is well aware of the limitations that rare disease patient numbers may place on the quality and quantity of evidence available for evaluation and already takes this into account. Moreover, the PBAC has demonstrated its flexibility in its decision making about cost-effectiveness.

2.3.3. The PBAC has indicated its receptiveness to consider developing additional guidance in its Submission Guidelines on submissions for rare disease.

2.3.4. Currently for a medicine to be eligible for consideration for listing on the Life Savings Drugs Program (LSDP) the PBAC must first have assessed the medicine as effective but not cost effective. The condition must then meet the criteria of the LSDP including that the medicine is life-extending.

2.3.5. The difference between whether a medicine qualifies for the LSDP or not may be marginal on clinical grounds. The PBAC has made recommendations for the PBS for some very high-cost medicines for relatively rare diseases. The evidence that medicines on the LSDP are lifesaving is frequently poor. On the other hand, there are medicines for rare diseases that are not lifesaving, but which have significant benefits to level of disability and quality of life.

2.3.6. The PBAC notes that the concept of rare disease is changing with increasing genomic sub-characterisation of more common diseases. This has the potential to further challenge the concept of a separate program for life-saving drugs for rare diseases.

2.3.7. The PBAC believes that the same purpose as the LSDP could be achieved through a PBS Section 100 program with specific criteria (as with other Section 100 programs). This removes the need for a second line of assessment and is highly unlikely to result in any disadvantage to patients or increase costs to government. It would also provide greater consistency in approval, pricing and ongoing monitoring. Such medicines are also more likely to be those potentially suitable for a managed access program.

2.4. Enhancing evaluation sharing with comparable reimbursement authorities.

2.4.1. The TGA has implemented several ways of sharing evaluations with similar regulators internationally and this has shortened the time to registration (and possibly the time to submission for registration in Australia).

2.4.2. The PBAC is interested in examining how similar types of sharing of health technology assessments could be implemented with other reimbursement authorities. Health technology assessments require more inputs that are country specific, such as local clinical practice, costs and availability of other therapies and supports, so there will always be a need for Australian specific assessments. However, there are elements that are likely to be very similar across countries.

2.4.3. A barrier to this is the confidentiality arrangements that companies have with different countries. While the PBAC understands the sponsor's reason for this in relation to pricing aspects, there would still seem to be substantial room for sharing of other aspects including economic modelling. Economic inputs would need to be adjusted to reflect country specific clinical practice, comparators and healthcare resource costs. It would appear to the PBAC that some global sponsors sometimes already use common models in their submissions to the PBAC.

2.5. Delegated authority to PBAC Executive.

2.5.1. The establishment of the PBAC Executive consisting of the Chair, Deputy Chair and Chairs of the Drug Utilisation and Economic sub-committees provides an opportunity for further efficiency in PBS processes.

2.5.2. This would be enhanced if decisions around some matters could be formally delegated to the PBAC executive.

2.5.3. This could include approvals for Section 19a exemptions for medicine shortages, changes in dispensed amounts, and changes to doses or minor changes to product content in the case of nutritional food products.

2.6. Alternative Submission Pathways

2.6.1. The PBAC observes that the current approach for consideration of medicines for listing on the PBS is largely reactive, and highly dependent on a sponsoring company being interested in a PBS listing.

2.6.2. The PBAC is aware of and concerned about situations where condition-specific clinical practice guidelines recommend medicines listed on the PBS but which are not PBS listed for those indications. This includes both on- and off-patent medicines. Such situations may lead to inequity in access to treatments.
2.6.3. The PBAC understands that companies may have limits to which they can initiate submissions for such purposes even where they may be supportive.
2.6.4. For some of these indications the medicine is approved for the indication in other jurisdictions, so it is not strictly speaking repurposing.
2.6.5. The PBAC notes the recent Department work on repurposing including an options discussion paper and the consultation on that paper. The PBAC would welcome further resolution of these matters.
2.6.6. The PBAC notes that while PBAC submissions may be made by other parties (e.g., clinical or patient groups) this is challenging given the PBAC requirements particularly without company sponsor engagement.
2.6.7. The PBAC sees benefit in an alternate mechanism to initiate submissions where there is an unmet clinical need and a potentially useful medicine.
2.6.8. Such an alternative pathway may include alternative sourcing arrangements (e.g., Calls for submissions for specific medicines) and would require resourcing a capacity to support the preparation of submissions.
2.6.9. Medicines for rare diseases includes off-patent medicines, some of which may not be financially interesting for a company to supply in Australia under current arrangements. The PBAC is aware of situations where supply has become problematic for some patients requiring these types of medications. It is essential that there is a mechanism to ensure availability of such medicines which may need to be different to the current submission-based mechanism and could include direct sourcing from an overseas supplier.

2.7. Ensuring supply of generic medicines.

2.7.1. The PBAC is aware of challenges to ensuring supply of generic medicines in the current market environment. It is important to note that generic medicines remain a substantial and critical part of the PBS for many patients and diseases.
2.7.2. The PBAC is aware of a number of situations where requests for deletion of medicines from the PBS have been driven by small demand for a product even though it may have an important place in current clinical practice. It is also aware of repeat requests for deletion, in effect repeated requests for price increases, claiming that the PBS price is not financially viable but where the size of the requested price increase is poorly justified.
2.7.3. The PBAC notes there may be a need for last resort mechanism to directly source providers for such medicines including potentially from suppliers not currently active in Australia to keep essential medicines available on the PBS.

3.0. Other matters

3.1. The PBAC notes that a number of submissions to the Inquiry raise issues about the extent to which the committee takes into account non-health care benefits and costs in assessing cost-effectiveness; the discount rates applied in

economic analyses especially in relation to vaccines and preventive medicines; the choice of comparator; and the use of real-world data. These, and other methodological issues would be better considered as part of a broader PBAC Submissions Guidelines review and the PBAC would be happy to do so. As with previous reviews, there would be wide consultation and an industry liaison working group and any changes to Government policy parameters would be taken into account.