DEAKIN UNIVERSITY

Submission to Senate Inquiry into PBS deferrals

INTRODUCTION

This submission to the Senate inquiry into PBS deferrals is made by a group of health economists from the Deakin Health Economics unit (part of the Faculty of Health) at Deakin University, Burwood, Victoria. All four signatories to this submission have extensive experience with the PBS process: Professor Rob Carter is a former Pharmaceutical Benefits Advisory Committee (PBAC) member; Liliana Bulfone, Patti Whyte and Sandra Younie are former senior evaluators of submissions (requesting public subsidy of pharmaceutical products) to the PBAC. We have all had the privilege of being able to attend meetings of the PBAC and its sub-committees and have either participated in or observed decision-making at these levels in practice. As a result of our experiences, we have a keen appreciation of how various factors contribute to decision-making in relation to subsidy of pharmaceuticals in Australia.

We were very pleased when the Senate referred the matter of recent deferrals of the listing of a number of pharmaceutical products on the PBS (despite a recommendation by the PBAC that the products should be listed on the PBS) to the Finance and Public Administration References Committee for inquiry as we have a number of concerns around the process used to determine which of the PBAC's recommendations should be implemented without delay and which of those recommendations can be indefinitely deferred.

This submission outlines our concerns under headings that approximately correspond with various terms of reference outlined for the inquiry.

THE DEFERRAL OF THE LISTING OF MEDICINES ON THE PBS THAT HAVE BEEN RECOMMENDED BY THE PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE

Until the Government's deferral of listing of seven medications on the PBS in February 2011, it was a relatively routine process that the Minister for Health and Ageing would accept the PBAC's recommendations for medications to be listed on the PBS following final negotiations of price and any risk-sharing agreements. It has been a very rare occurrence for a product that received a positive recommendation from the PBAC not to be listed on the PBS. In fact, we know of only two such occasions in the history of the PBS when Federal Cabinet has intervened to reject a PBAC recommendation – one was in the case of nicotine patches and the other was in the case of Viagra®. In both of these cases, the forecast expenditure on the medications was high and concern about affordability of the medications in the short term appears to have been the main motivation for not proceeding with a listing. In the case of Viagra®, although the PBAC recommended listing of Viagra® on the PBS for certain patients, the recommendation was accompanied by a clear warning from the PBAC that the listing of this product on the PBS may cause a significant budgetary impact on the PBS. In the case of nicotine patches, although it was accepted that the benefits in the long term justified the cost of the patches, the patches were considered to not be affordable in the short term due to the numbers of smokers likely to access the treatment under the PBS.

We understand and agree with some of the claims made by the Hon. Nicola Roxon MP, Minister for Health and Ageing, in her opening address to a roundtable conference co-hosted by an alliance of the Consumers Health Forum, a number of other peak health consumer organisations, the Australian Medical Association, Medicines Australia and the Generic Medicines Industry Association in Melbourne which was held on Friday 29 April 2011[1]. We understand and agree that there is a limit to how much money a government can spend on pharmaceutical products and that funds directed to pharmaceuticals have an opportunity cost (i.e., there are always competing priorities that need to be balanced and managed). We also appreciate that the decision about how public funds should be allocated rests with Government. We therefore wish to make it clear that we don't have any issues with the principles expressed that government may need to prioritise spending within and across various government programs. However, we have a number of concerns around the process

that the Government is using to prioritise the PBAC's recommendations into a list of medications that should be listed on the PBS without delay and a list of medications where listing on the PBS can be delayed.

We are concerned that the Government's action of deferring the PBS listing of some medications that were all recommended for listing on the PBS by the PBAC demonstrates a lack of understanding of what a PBAC determination of "acceptable cost-effectiveness" means. Targin® was recommended for listing for management of chronic disabling pain on the basis of cost-effectiveness compared with oxycodone controlled release tablets. Gilenya® was recommended for listing for the management of patients with multiple sclerosis on the basis of an acceptable cost-effectiveness compared with interferon beta-1a. Tobi®, an antibiotic to treat lung infection in patients with cystic fibrosis, was recommended for listing on the basis of acceptable cost effectiveness compared with placebo. Pradaxa®, an anticoagulant, was recommended for listing to prevent stroke in patients with atrial fibrillation who are at moderate-severe risk of having a stroke on the basis of acceptable cost effectiveness (presumably compared with warfarin or doing nothing - the current management options). Recommendations based on cost-effectiveness mean that the PBAC has accepted that the drug is associated with superior outcomes compared to current treatment and the additional benefits generated justify the additional cost of this drug compared with current treatments. Lopert et al, 2002[2], report that the World Bank has suggested that health-care interventions may be considered acceptably cost effective if they buy a year of life at full health for less than the national per capita GDP. Presumably, the rationale for the nomination of this threshold is that that adding a year of life at full health to a person should, on average, translate to an increase in GDP (by the average national per capita level). The threshold at which PBAC determines a drug is acceptably cost-effective is approximately in line with current per capita GDP[3] (which stands at approximately $55,000[4]). Thus, it could be argued that when the PBAC makes a determination that a drug is cost-effective, this means that it can be expected that the returns (in terms of impact on the overall economy from gains in the health of the population) justify the investment. Although the decision to defer two of these drugs, Gilenya® and Tobi® has recently been reversed, the decision to defer the listing of drugs that have been accepted to be superior to the alternatives currently available and that have been accepted as representing value for money, is therefore perplexing.

THE CRITERIA AND ADVICE USED TO DETERMINE MEDICINES TO BE DEFERRED

Our greatest concern is that a set of non-disclosed and potentially arbitrary set of criteria (if any exist!) are being used to divide a list of positive recommendations made by PBAC into two groups: (a) recommendations that should be implemented without delay; and (b) recommendations whose implementation be deferred.

The criteria that have been used to divide the list of PBAC's recommendations into those should be implemented without delay and those that can be delayed have not been articulated by the Government. Furthermore, the credentials of those making these determinations (in terms of their expertise and experience that would qualify them to make decisions about differential allocation of funds) have not been disclosed. When asked by a Senate Estimates Committee what criteria had been used to determine which of the drugs recommended for listing on the PBS should be deferred, Ms Halton (Secretary for the Department of Health and Ageing) replied "I do not think we can provide any such thing, because I do not believe it exists"[5]. As the Government's process resulting in deferral of the listing of selected medications (despite recommendation for inclusion on the PBS by the PBAC) is not transparent, the possibility that the process is capricious and that arbitrary criteria are being used to select medications for deferral cannot be excluded. The lack of an articulated basis upon which decisions to defer are made leaves the government open to criticism of poor governance processes. Poor governance processes give rise to perceptions that decisions are being made on the basis of influence or pressure in contrast to the independent scientific basis that underpins PBAC decision-making. The Government has left itself without any basis on which to refute criticisms that cabinet decisions are unfair or biased. Clearly, an erosion in the faith we have in government to make decisions in our - the

public's interest - is an undesirable outcome for everyone that desires a healthy functioning government and health care system.

We examined the original list of seven deferrals of medications recommended for listing on the PBS by the PBAC in November 2010 and it does not appear that there is any consistent basis for determining when it is acceptable to defer implementation of a positive PBAC recommendation.

As drugs recommended for listing on the basis of cost-minimisation were included in the list of deferred items (e.g., Duodart® for men with enlarged prostates [a combination product containing dutasteride and tamsulosin that will substitute for two products - dutasteride and prazosin] and Symbicort® which can be used by patients with lung conditions [and which will substitute for Seretide®]), it cannot be claimed that a lack of cost-offsets or presence of unacceptable overall financial implications are the drivers of decisions to defer. In the case where recommendations for listing of a medication on the PBS has been made by the PBAC on the basis of cost-minimisation, the PBAC has accepted that, taking a whole of health system perspective, the drug is no more expensive than the therapy most likely to be replaced (the comparator) such that there should therefore be no overall fiscal impact of making the drug available. Another example demonstrating that decisions to defer could not have been based on budget impact is the case of Synarel®. In this case, a Senate estimates committee was told by a Department of Health and Ageing official (Adriana Platona) that Pfizer, the manufacturer of Synarel® (used for fertility treatment) had been asked to submit an application to have the product included on the PBS to stimulate price competition in this market[5].

It also does not appear that decisions to defer were made on the basis of clinical need for a product or on the basis that alternative treatments are already available. Products for which there is an undisputed clinical need and for which equally effective alternatives are not available were also deferred (e.g., the drugs listed on the basis of acceptable cost-effectiveness discussed above [i.e., drugs accepted as being superior to currently available therapies] and in the case of Invega Sustenna® and Risperdal Consta®, both antipsychotic agents that have a different mode of action compared with antipsychotic agents that are already available). It is important to note that a recommendation for inclusion of a drug on the PBS on the basis of non-inferiority compared with another drug that is already available (as occurred in the case of these antipsychotic drugs) does not mean that the new drug is interchangeable with the drug that is already available. For example, although the extent of benefit in a population may be similar for patients treated with two drugs (which is the basis for a recommendation of listing on the basis of non-inferiority [such that the new drug is recommended for listing at the same price as a currently available drug), this does not mean that the drugs will result in identical outcomes on an individual patient basis. That is to say that even if the same proportion of patients in a population "respond" to a treatment and the same proportion experience adverse events, it does not necessarily mean that it will be the same identical patients experiencing benefits and adverse effects in each instance. Patients who may not respond to one drug may respond to another and patients who experience adverse effects with one medication may not experience those effects if they take a different medication. The variation in individual response typically arises as a consequence of differences in the chemical, pharmacological and clinical properties (such as differences in the profile of adverse events associated with each treatment) across the drugs recommended for inclusion on the PBS. These differences can have a material impact on outcomes for individual patients and are therefore taken into consideration by physicians when deciding which therapy is most suitable for an individual patient.

It also does not appear that the magnitude of the incremental cost-effectiveness ratio (i.e., the extent of additional cost relative to the number of years of life at full health [quality-adjusted life-years] associated with a drug) is the basis for the decision as drugs with relatively low incremental cost-effectiveness ratios (e.g., Prevenar-13) were deferred.

In her opening address to a roundtable conference co-hosted by an alliance of the Consumers Health Forum, a number of other peak health consumer organisations, the Australian Medical Association, Medicines Australia

and the Generic Medicines Industry Association in Melbourne which was held on Friday 29 April 2011[1], the Hon. Nicola Roxon MP, Minister for Health and Ageing, suggested that a good example of competing priorities that needed to be balanced against the listing of medications on the PBS was the Bowel Cancer Screening Program, a program with an associated cost of $137 million. The question was asked "Do we sacrifice this program to cover adding new drugs to the PBS?". This statement raises another concern for us - the lack of clarity over the process (if any!) used to decide how funds should be allocated across competing priorities (pharmaceutical interventions versus other health interventions). In contrast to pharmaceutical products that are subject to rigorous assessment by the PBAC to determine whether investment in a drug is justified by the returns generated by the drug, other health interventions (e.g., preventative health interventions) are not subject to such rigorous assessment. A health economist would argue that if they had a choice about whether a limited number of dollars should be spent on Drug A or Intervention B, the health economist would advise that a consideration of which one generates best value for money should drive that decision (i.e., resources should be directed to the intervention that provides the greatest health benefit for a given expenditure). Essentially, the decision is one of which intervention provides the greatest return for the investment made. The incremental cost-effectiveness ratio is accepted as providing an indication of value for money. In conducting an analysis of incremental cost-effectiveness, the incremental cost associated with the intervention is compared with the incremental health gain (e.g., measured in terms of years of life at full health [quality-adjusted life-years]) produced by that intervention compared with the currently available alternative. However, an issue that arises is that the cost-effectiveness of many health interventions to which public funds are diverted are unknown. For example, the Bowel Cancer Screening Program has not been through an assessment process as thorough as that of the PBAC's. There has been ample opportunity for collection of relevant data to permit such an assessment to be conducted but, to date, no such assessment of the pilot program has been released or reported by the Government. When the Government chooses to defer the funding of a drug whose cost-effectiveness is known and accepted and direct funds to a program whose cost-effectiveness is unknown, it is potentially using available funds inefficiently. Rather than destabilise one of the most highly regarded assessment processes in the world, the government should be encouraging generation of better evidence for the competing priorities to inform whether resources should be allocated to these areas or not. If every decision about whether to allocate funds to a health care intervention were made on the same basis as used by the PBAC, the sustainability of our healthcare system could be ensured.

CONSEQUENCES FOR PATIENTS OF DEFERRALS

It is self-evident that deferring the listing of medicines on the PBS that have been accepted by an independent statutory body (PBAC) to be effective, safe and cost-effective will result in denial of access to effective therapy for many patients. The deferrals also have the potential of introducing inequities in access to medicines that have been accepted as being effective, safe and cost-effective by the PBAC. This is particularly the case when the Government, as well as appearing to capriciously defer the listing of medications, also appears to change its mind capriciously in response to media pressure (as has occurred with the reversing of the deferrals for Erbitux® and Gilenya®)

The fact that a pharmaceutical manufacturer may no longer be able to be confident that once a drug has a positive recommendation from the PBAC that it will be made available on the PBS introduces additional uncertainties and risk for manufacturers. As an example, consider the case of Synarel® – the manufacturer of the product was asked by Government to submit an application to have their product listed on the PBS, they paid $12,500[5] to have their submission assessed by the PBAC (in addition to the costs of assembling the submission itself), they had their drug recommended by the PBAC but then have had the listing deferred indefinitely by Cabinet. How can industry or the public have any confidence whatsoever in this process? The hurdle of gaining a positive PBAC recommendation is a substantial barrier to market access in itself. Furthermore, manufacturers are, in most cases, required to pay substantial sums of money to have their drug considered by the PBAC. To then have to negotiate a new hurdle (the approval of the drug by Cabinet

according to some undisclosed set of criteria) may mean that the consideration of the benefit of having a drug available on the PBS is outweighed by the costs and risks of achieving a PBS listing such that, over time, manufacturers may choose to not engage with the process of trying to make drugs available on the PBS in Australia such that drugs may be available in the private system but not the public system. This will be detrimental to Australian patients as they will have to bear the full cost of drugs and, in many cases, it is likely that the costs associated with a drug will put the drug out of reach altogether. For drugs with small markets where costs to patients are likely to be prohibitive, manufacturers may not even make the drug available in the private market.

Compliance with the intent of the Memorandum of Understanding between Medicines Australia and the Minister for Health and Ageing

A requirement for Cabinet consideration of products that cost more than $10 million in any of the first four financial years of being listed on the PBS was first introduced in 2001. This led to substantial delays in the listing of some products on the PBS. Medicines Australia estimated that the requirement for Cabinet consideration has led to delays of up to ten months. In return for various concessions by Medicines Australia in a memorandum of understanding (MOU) signed by Medicines Australia and the Hon. Nicola Roxon MP, Minister for Health and Ageing (e.g., acceptance of price reductions for drugs where a generic is available and agreement to comply with price disclosure methodologies), the Minister (on behalf of the Commonwealth of Australia) undertook to make "best endeavours" to implement a maximum time frame of six months for consideration of a drug recommended by the PBAC by Cabinet. The use of "deferrals" appears to us to be a sneaky way of avoiding adhering to the undertakings made under the MOU. It appears to us that the term "deferral" is really a euphemism for rejection because there is no commitment from the Government as to exactly when the deferred drugs will be re-evaluated. The Government has simply said it will "reconsider deferred applications when fiscal circumstances change."

It is our opinion that the lack of adherence to the spirit of the MOU is short-sighted as it is possible, if not likely, that the failure of the government to act in good faith in this instance will have repercussions for future negotiations between the pharmaceutical industry and government. Furthermore, it is possible that the failure of the government to uphold the spirit of the MOU will have flow-on effects for negotiations of agreements between government and other industries.

Concluding remarks

The PBS listing process is a rigorous and effective process that is highly regarded around the world. The PBS should not be viewed only as a cost to Government; it is an investment in the health of Australians. Medicines can save the health budget money through reduced need for other clinical interventions but, more importantly, medications can lead to improvements in quality of life and survival which can provide benefit to the economy through increased participation in the workforce and engagement with society. The PBAC is well qualified to assess whether the costs associated with a drug are justified by the benefits that the drug generates. In our opinion, the addition of further hurdles to the process (e.g., by requiring cabinet review of all positive PBAC recommendations) and the politicisation of this process is unacceptable and is to be avoided. The introduction of a process that divides PBAC recommendations for listing of medications on the PBS into those that should be implemented immediately and those that can be "deferred" (indefinitely) is a poorly considered, short-sighted policy decision that will impact on the unwell - some of the most vulnerable in our society. We urge the Government to rethink this policy.

Signed:

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