Committee Secretary,
Senate Finance and Public Administration Committees
PO Box 6100
Parliament House
Canberra ACT 2600
Australia

Dear Committee Secretary,

Thank you for the opportunity to provide a submission on the Senate Committee's Inquiry into the Government's Administration of the Pharmaceutical Benefits Scheme (PBS). This submission provides recommendations to move forward with an urgent review of the PBAC/Cabinet processes in approving drugs under the Pharmaceutical Benefits Scheme (PBS). This is an important area of the Government's administration of the PBS. My submission relates to mechanisms to address the Terms of Reference for the Senate Inquiry in light of stakeholder arguments documented in the media, along with recent international discussions of related issues at the International Health Economists Association (IHEA) Congress I attended in Canada during July 2011. An in-depth discussion of the various issues raised by stakeholders have been well documented elsewhere[1] [2] [3] [4] [5] [6] [7] [8] [9] and will not be repeated here. The UK's National Institute for Health and Clinical Excellence (NICE) is an international world leader in methodologies and processes related to drug approvals. Key insights from the experience of NICE are also used to develop the recommendations in this submission. Section 1 discusses the NICE's initiatives. Section 2 includes the recommendations for the proposed review in light of insights arising from NICE's work, the IHEA discussions and the views of key stakeholders in the PBS deliberations.

1. National Institute for Health and Clinical Excellence (NICE): UK

The National Institute for Clinical Excellence (NICE) was established in 1999. It was renamed the National Institute for Health and Clinical Excellence in 2005. Health Technology Assessments (HTAs) are used to develop guidance for the National Health Services (NHS) on the use of health technologies. NICE provides national guidance on good health, prevention and treatment. NICE has produced three types of guidance including clinical guidelines, technology appraisals and interventional procedures. In 2005 NICE assumed responsibility to develop guidance for public health interventions and programs. *NICE's remit is to consider both cost effectiveness and clinical effectiveness in developing its guidance. From January 2005 technology appraisals were supported by mandate and the NHS in England and Wales are legally obligated to provide funding for drugs and treatments recommended by NICE. If NICE's guidance supports the availability of a technology by the NHS for specific patient groups, then health care organisations are obligated to implement the recommendations within three months from the guidelines' issue date.* NICE is also involved in topic selection for reviews (Drummond and Sorenson, 2009)[10].

Further details about NICE's functions and approach are included in Attachment 1, which is a journal article entitled *'Nasty or Nice? A Perspective on the Use of Health Technology Assessment in the United Kingdom'* by Professor Michael Drummond and Corinna Sorenson (2009). Some key issues in that article are highlighted below as they are considered instructive for Australia's current Senate Inquiry into the PBS. NICE's methodological guidelines embody several features of the Australian and Canadian guidelines. The focus below covers the perspectives on costs, subgroup analyses and probabilistic sensitivity analyses (PSA), transparency, stakeholder involvement, pricing, QALYs and social values, cost effectiveness thresholds and the impact of treatment/patient type. Some of these methodological issues were also raised during the International Health Economists Association (IHEA) Congress and Pre-Congress sessions in Canada that I attended during mid July 2011 Some of the PBS issues related to the Australian Senate Inquiry were discussed during those sessions. These insights have influenced this submission. I turn now to a discussion of insights from NICE as identified by Drummond and Sorenson (2009).

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[1] Kerin J (2011) 'Doctors lash out on health and drug reforms' *The Australian Financial Review* July 21 2011 pg 9
[2] Dunley S (2011) 'Big Pharmaca Leads Revolt: Warning PBS row will stall drugs' *The Australian* Page s1 and 8. 20 July
[3] Van de Berg L (2011) 'Outrage as Government keeps vital new medicine off PBS: Sick face drug delay'. *Herald Sun* pg 4 20 July
[4] Hagan K (2011) 'Anger over drug subsidy delays: Patients at risk companies claim' *The Age* pg 6 22 July
[5] Dunley S (2011) 'Paracetamol a pain for the PBS' *The Australian* 26 July pg 1 and 6.
[6] Connors E (2011) 'Consumers, companies unite on PBS deferrals' *The Australian Financial Review* 26 July pg 7.
[7] Dunley S and Franklin M (2011) 'Doctors should select PBS drugs, say marginal voters' *The Australian* pg 7 29 July
[8] Connors E (2011) 'Anger flares over unsubsidised drugs'. *The Australian Financial Review* 29 July pg 7
[9] http://www.aph.gov.au/senate/committee/fapa_ctte/pharma_benefits_scheme/submissions.htm - Submissions to the Senate Inquiry.
[10] Drummond, M and Sorenson C (2009) Nasty or Nice? A Perspective on the Use of Health Technology Assessment in the United Kingdom *Value in Health* Vol 12 – Supplement 2, S8-S13. International Society for Pharmacoeconomic and Outcome Research (ISPOR)

Perspectives on costs. In the primary analysis, costs include the NHS and personal social services costs only. NICE's remit is to advise on the best use of the NHS budget. However, the perspective has been broadened to consider costs relating to other public sector budgets, for the appraisal of public health programs. Hence, a public health appraisal of an education program to reduce substance abuse can consider criminal justice cost reduction. However, the technology appraisal of drug maintenances for heroin addicts could not. Dummond and Sorrenson (2009) [1] describe this situate as 'perverse'. I agree with that description.

Subgroup analysis NICE is keen to analyse costs and effects for patient sub-groups even if the original clinical studies were not powered to assess the differences. NICE considers results of 'uncertainly' around the estimates (Drummond and Sorenson, 2009) .

Probabilistic Sensitivity Analysis (PSA). NICE's insistence on PSA has been criticized given computational difficulties and the length of time related analyses can take. NICE has commissioned independent analyses by independent assessment groups. The resource intensity of studies may restrict the number of technology appraisals to be performed and NICE has established the Single Technology Appraisals (STA) Program which requires less time, approximately 39 weeks. The time period is lower than the minimum 54 weeks for larger assessments but appeals are delaying the time frame. STAs are mainly applied to drugs and the program is similar to that in Scotland.

Transparency Both the Appraisal Consultative Documents (ACDs) and Final Appraisal Determinations (FADs) are posted on the web although *the Parliamentary Health Select Committee's Review of NICE found that NICE should open up the Technology Appraisal Committee hearings to the public where the evidence is being discussed. This is currently under review*. The Parliamentary review also found that NICE should make executable version of the assessment group models available to manufacturers (Drummond and Sorenson, 2009).

Stakeholder Involvement *All stakeholders – manufacturers, professional groups, patient organisations and the NHS can comment on the Appraisal Consultation Document and Final Appraisal Determination. If they are unhappy with the FAD, stakeholders can appeal. Approximately 30% of NICE's decisions have been subject to appeals. Recently, there are signs appeals may rise with Single Technology Appraisals (STDs)* (Drummond and Sorenson, 2009).

Unresolved Issues

Although NICE is 'arms length' there are accusations it is following the government's agenda. However, there are very few examples of government actions impinging on NICE's work. One exception is the government interference when NICE's decision did not issue positive guidance on the use of beta interferon for multiple sclerosis. The government brokered a risk-sharing scheme with the manufacturers. Government can retain part of the expenditure on beta interferon if the long term benefits are not as favorable as the manufacturers claim. Recently, the Office of Fair Trading argued that the Pharmaceutical Price Regulation Scheme should be abandoned in favor of a 'value based pricing'. If this was implemented, there may be a role for NICE in the process and NICE could then be seen as the government's price negotiator for drugs (Drummond and Sorenson, 2009).

QALYs and Social Values The QALY does not capture all the elements of social value relevant to decisions about the allocation of health care resources. *QALYs are conceptualized of equal value irrespective of who receives it. 'Society' if consulted would not apply this principle (eg) the public may value a QALY for a severely ill patient higher than to one in relatively good health. The Citizens Council has been used by NICE to explore this. The seriousness of a patient's condition is seen as a factor that should be taken into account* (Drummond and Sorenson, 2009).

The cost effectiveness threshold Three criticisms, in general have been raised in debates about NICE's use of a threshold, viz *"1) there should not be a threshold 2) the threshold has been set at the wrong level or is arbitrary; and 3) different thresholds should apply, depending on the nature of the treatments or patient populations being studied"* (Drummond and Sorenson, 2009 pg S11). The authors rightly point out that a more sensible question, than the first raised above, is whether the cost effectiveness threshold (or thresholds) used should be explicitly stated. With regard to the range at NICE, interventions with an incremental cost lower than 20,000 pounds per QALY have a higher probability of being funded. Those exceeding 30,000 pounds have a lower probability of funding. Equity issues may support scenarios higher than 30,000 pounds. NICE's threshold range is on the high side (See Drummond and Sorenson, 2009 for a review). Research is underway to determine the feasibility of estimating a monetary value of a QALY from a societal perspective. *The threshold could differ depending on the treatment being evaluated or the patient population being investigated*. If the QALY does not adequately capture all of the relevant elements of social value, then a single threshold may not make sense eg drugs for rare diseases such as orphan drugs. Even if they do not appear cost effective, society may prefer to make them

available because they may related to many diseases that are life threatening and would be unfair not to make them available. Alternatively, valuing rarity per se may not be preferred because resources could be denied to patients with more common diseases which could be equally serious (McCabe et al 2005[11]; Drummond and Sorenson, 2009).

Further research is required on whether QALYs should be weighted equally and whether society values attributes of health care that cannot be easily incorporated in the QALY framework. Severity of illness or availability of alternative treatments could be relevant. However there is little evidence that these feature in NICE's decision making. However, NICE has indicated a possibility of a higher cost effectiveness threshold around 10 times higher the existing threshold may have to be applied for ultra orphan drugs (ie drugs for conditions with a prevalence of less than 1 in 50,000). Recently NICE has indicated consultation on whether there should be a higher threshold on the evaluation of treatments in the final months of life (eg mestastic cancer). (NICE, 2006[12]; Drummond and Sorenson, 2009).

2. Recommendations

1. An urgent review be held of the processes the PBAC and the Cabinet in deliberations for approving drugs for the PBS. The review should be finalized by November 2011 and could involve an international health economics expert with experience in the area. I recommend that Professor Michael Drummond of York University be invited to participate in this review, if he is available, given his extensive experience of the UK system and his involvement with NICE's deliberations.

2. Some key insights arising from the NICE deliberations, and at the International Health Economists Association (IHEA) Congress in Canada in May 2011, should be considered in the review process recommended in 1) above and could include:
    a. **Perspectives on costs**: Implications of the broadening of perspective beyond drugs to include other health programs in deliberations by governments in the budget context, which can be perverse. *How can the evaluation methodologies in Australia be improved to ensure equity where this process must proceed given a political decision to implement such a policy?*
    b. **Transparency issues** in the UK whereby the Parliamentary Health Select Committee's Review of NICE found that NICE should *open up the Technology Appraisal Committee hearings to the public* where the evidence is being discussed. *If appropriate, what mechanisms can enable public hearings in Australia at various stages of the evaluation process?*
    c. **Stakeholder involvement** in commenting on the Appraisal Consultation Document (ACD) and Final Appraisal Determination (FAD). If they are unhappy with the FAD, stakeholders can appeal. Approximately 30% of NICE's decisions have been subject to appeals. *Should there be appeal processes in Australia? At what stage would an appeal process be appropriate?*
    d. **QALYs and social values** QALYs are conceptualized of equal value irrespective of who receives it. However, the public may value a QALY for a severely ill patient higher than to one in relatively good health. The Citizens Council has been used by NICE to explore this. The seriousness of a patient's condition is seen as a factor that should be taken into account. *Should a Citizens Council be established in Australia to explore weightings QALYs?*
    e. **The cost effectiveness threshold** The threshold could differ depending on the treatment being evaluated or the patient population being investigated. Should QALYs be weighted equally? Severity of illness or availability of alternative treatments could be relevant. NICE has indicated a possibility of a higher cost effectiveness threshold around 10 times higher the existing threshold may have to be applied for ultra orphan drugs. NICE has undertaken consultation on whether there should be a higher threshold on the evaluation of treatments in the final months of life (eg mestastic cancer). *Should explicit thresholds be established for varying diseases and/or severity of illness? Could Australia lead the way in this area? Could the proposed Citizens Council undertake this in consultation with leading experts?*

3. Ensure the frameworks developed by the review are legally consistent with *Part VII of the National Health Act 1954 as amended*, given it is the legislative authority for the PBS.

[11] McCabe C Claxton, K Tsuchiya A (2005) Orphan drugs and the NHS: should we value rarity? BMJ 331: 1016-19 (cited in Drummond and Sorenson 2009).
[12] National Institute for Health and Clinical Excellence (NICE) (2006) *Appraising Orphan Drugs*, London: NICE Cited in Drummond and Sorenson 2009)

4. Consideration of both the current PBAC and Cabinet deliberations in drug approvals and whether further changes should be made.

5. Impact of government policy on drug companies and patients whereby ***all*** positive recommendations by the PBAC and the Pharmaceutical Benefits Pricing Authority (PBPA) with a financial impact on the Commonwealth Budget are under consideration by Cabinet.

6. Implications of Government policy whereby PBS, Life Saving Drug Program and National Immunization Program listings are *subject to the **same** government scrutiny as other spending measures **across the health portfolio and government generally***.

7. Desirability of formal, transparent and timeline rules to underpin the government's assertion that *'those listings which have been deferred in the prevailing fiscal environment remain eligible for future consideration when fiscal circumstances permit'* (Department of Health and Ageing pg 9).[13]

8. Implications of current government policies announced in February 2011 on the role of Cabinet on drug approvals with regard to *the impact on financial loss by drug companies and the possible decline in undertaking clinical trials in Australia*.

9. Consideration of the estimated growth of the PBS of 7.7% and 6.5% in 2010-11 and 2011-12 respectively, as reported on the Portfolio Budget Statement 2011-12 and likely impact of the government's revised reform measures on patients and drug companies.

10. Consideration of the government's *stated* current policy focus on listing drugs that treat serious or life threatening conditions where there are no alternative treatments on the PBS.

11. Impact of the implementation in January 2011 of the Managed Entry Scheme and Parallel Processing of submissions to the PBAC and TGA.

12. Impact of the situation whereby PBAC considers PBS and NIP listing submissions on safety, clinical and cost effectiveness with ***no*** additional formal criteria for decision making about deferring the listing of drugs - The Department does not provide advice to the Cabinet about which of the PBAC recommended drugs should be deferred or funded. The Government considers whether the listing provides expenditure savings and other information the PBAC considered.

13. Desirability of developing transparent and effective *Evaluation Criteria* for use by Cabinet in considering the PBAC recommendations to enable equity.

14. Implications of the government's imperatives to return to budget surplus by 2012-13 and whether alternative drug policy directions could achieve the aim with greater equity that the current system.

15. Broad stakeholder inclusion in the review including eg – clinicians, consumers, drug companies, Medicine Australia, other stakeholders such as AMA, RACGPs, Research Australia.

16. Reporting mechanisms to ensure transparency of the PBS listing process

17. Mechanisms to enable the re-consideration of the review process for deferrals, existing and new drugs.

18. Implications of the role of Cabinet in the approval process and the Memorandum of Understanding signed in May 2010 and National Medicines Policy.

[13] Department of Health and Ageing (2011) *Submission to the Senate Finance and public Administration References Committee Inquiry into: The Government's Administration of the Pharmaceutical Benefits Scheme*. 15 July 2011.

19. Impact of the new policies on patients and drug companies of the expected delay in the current review of medicines for areas such as cancer, diabetes, cardiovascular and mental health.

20. Impact of PBS growth estimates shown in the Inter Generational Report 2010.

21. An analysis of the impact of the changes to the previous $10m per annum threshold for the Cabinet Review process. This should consider the recommendations about the threshold level by the previous Senate Community Affairs Reference Committee Inquiry into Consumer Access to Pharmaceutical Benefits Report in November 2010 and the Productivity Commission's recommendations about the threshold in the Annual Review of Regulatory Burden on Business in 2008 as identified by Medicines Australia[14].

22. The reports to the Access to Medicines Working Group on PBS Drivers and PBS Horizon Scanning for the 20 August 2011 meeting.

23. Thorough analyses of options for future processes including one option of the government reverting to processes existing prior to 2011.

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Dr Antioch is Deputy Chair, Guidelines and Economists Network International (GENI). GENI includes Board members from OECD, World Bank, Australia, UK, USA, Canada, China, NZ, Sweden and Denmark. GENI's Terms of Reference are to drive international mechanisms to integrate Clinical Practice Guidelines, clinical and cost effectiveness evidence into national decision making and clinical practice. It aims to forge links with national bodies that set standards, regulation and funding processes such as governments and insurance bodies. Dr Antioch held Ministerial appointments to the Principal Committees of the NHMRC – the National Health Committee and Health Advisory Committee from 2003 to 2009. As Principal Adviser to the CEO of Bayside Health (now Alfred Health) to 2005, and as Principal Management Consultant for Western Health until 2007, she led the integration of cost effectiveness and clinical evidence into clinical practice across all hospitals of the two large Victorian hospital networks. She was invited by the Australian Government Department of Health and Ageing during 2009 to participate in the focus groups to develop the framework and recommendations for the Review of Health Technology Assessment in Australia by the Federal Health Minister and Federal Minister for Finance and Deregulation. She is Associate Editor of the Cost Effectiveness and Resource Allocation Journal and Editorial' Advisory Board Member, Open Pharmacoeconomics and Health Economics Journal

[14] Medicines Australia (2011) Submission to the *Senate Finance and public Administration References Committee Inquiry into: The Government's Administration of the Pharmaceutical Benefits Scheme*.