INNOVAL HC
Institute for Innovation & Valuation in Health Care

Institut für Innovation & Evaluation
im Gesundheitswesen

An der Ringkirche 4
D-65197 Wiesbaden

Tel: +49 (611) 4080 789 10
Fax: +49 (611) 4080 789 99
michael.schlander@innoval-hc.com
www.innoval-hc.com

PHARMACEUTICAL BENEFITS ADVISORY COMMITTEE (PBAC) GUIDELINES REVIEW 2016:

AN INTERNATIONAL PERSPECTIVE

EXPERT STATEMENT

[*Focus on Selected Topics*]

prepared **by**

Michael Schlander

MD, PhD, MBA
Professor of Health Care & Innovation Management
Universities of Ludwigshafen & Heidelberg / Germany

Chairman & Scientific Director
Institute for Innovation & Valuation in Health Care

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Kathryn Glase and Nathan Walters, 1-5 Khartoum Rd, North Ryde NSW 2113 Australia
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Introduction

The Australian Minister for Health, Sussan Ley, approved a review of the *Guidelines for Preparing Submissions to the Pharmaceutical Benefits Advisory Committee* (the Guidelines). The review is carried out by an external contractor, the Adelaide Health Technology Assessment (AHTA), and is supported by a Guidelines Review Steering Committee consisting of representatives from the Pharmaceutical Benefits Advisory Committee (PBAC) and its sub-committees, stakeholders from industry, HTA experts, and the Australian Government Department of Health. The new Guidelines, version 5.0, are expected to be "of the ***highest quality*** and […] continue to ***reflect best international practice***."[1]

Stated Objectives

The Guidelines are used to prepare (major) submissions to the PBAC so it can assess whether a product should be included on the Pharmaceutical Benefits Scheme (PBS).

The Guidelines [are intended to] help ensure that Australians have ***access to safe, clinically proven and cost-effective medicines as soon as possible***[1], while ***safeguarding the sustainability*** of the PBS so it can benefit future generations. The Guidelines are now being reviewed to ensure assessment processes are ***consistent, transparent***, and continue to ***address contemporary methodological issues*** associated with the PBS.

Context I: Medicare and the PBS

The PBS is part of Australia's National Medicines Policy. The Policy's objective is "to meet medication and related ***service needs***, so that both optimal ***health outcomes and economic objectives*** are achieved."[2] The policy has been described to rest on "four central pillars: medicines meeting appropriate standards of ***quality, safety, and efficacy***; ***timely access*** to medicines that Australians need, ***at a cost individuals and the community can afford***; the ***quality use*** of medicines; and the maintenance of a responsible and viable ***drug industry***" (Lopert, 2009).

The assessment against the first set of objectives (i.e., efficacy, safety, and quality) falls under the remit of the Therapeutic Goods Administration (TGA) within the Department of Health, which (if its evaluation is positive) grants regulatory approval (marketing authorization) for a medicinal product. The TGA determines the conditions of use, under which the product may be dispensed as a private prescription, and the patient will have to pay the full cost. In order to be subsidized by the PBS, the product will have to undergo a subsequent positive evaluation by the PBAC, followed by price negotiations between the manufacturer and the Pharmaceutical Benefits Pricing Authority (PBPA).

Context II: The role of PBAC

Following the official website of the Department of Health, "the PBS provides ***timely, reliable and affordable access to necessary medicines*** for Australians."[2] PBAC acts as a gatekeeper regulating entry to the PBS. According to the National Health Act 1953, Sect 101, its functions include to "[…] give consideration to the ***effectiveness and cost*** of therapy involving the use of the drug, preparation or class, including by ***comparing*** the effectiveness and cost of that therapy with that of ***alternative therapies***, whether or not involving the use of other drugs or preparations."[3]

Context III: Australia's pioneering role in formal HTAs

Australia was the first jurisdiction to introduce explicit consideration of "***value for money***" as a prerequisite for formulary listing within its national pharmaceutical reimbursement program, i.e., the

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[1] Source: *www.pbs.gov.au/info/reviews/pbac-guidelines-review*; last accessed March 28, 2016. Certain parts of text were added or modified, and these parts are identified by the use of [closed] brackets; other parts of text were highlighted by using ***bold italics*** not existent in the original quoted here.

[2] Department of Health and Ageing, 1999.

[3] Excerpt from *http://www.austlii.edu.au/au/legis/cth/consol_act/nha1953147/s101.html*, last accessed March 28, 2016.

PBS, in the early 1990s. The Australian approach served as a role model for many other jurisdictions that later introduced official Health Technology Assessments (HTAs), often including formal economic evaluation, as a hurdle to be passed prior to reimbursement. The inauguration of the new approach in Australia "was not intended as a cost-containment mechanism per se, but rather as a way of ensuring that drugs added to the national formulary reflect value for money" (Lopert, 2009).

The criteria applied by PBAC for evaluation are delineated in detail in the **PBAC Guidelines**. These criteria are of utmost relevance because a new drug cannot be added to the PBS formulary in the absence of a positive recommendation by PBAC. (However, the Minister for Health may decline a recommendation for listing by the PBAC.) The Guidelines may thus be usefully discussed in light of international experience and recent developments in health economics as an academic discipline.

International Standards I: Academic recommendations

Against the background of the worldwide heterogeneity and the dynamic processes driving the adoption of HTA as a decision support tool, an international group of health economists (Drummond et al., 2008) proposed a set of 15 key principles for the conduct of HTAs for resource allocation decisions: "1. HTAs should have explicit and relevant goals and scope. 2. HTAs should be unbiased, rigorous and transparent. 3. HTAs should include all relevant technologies. 4. HTAs should have a clear system for setting priorities. 5. HTAs should incorporate appropriate methods for assessing costs and benefits. 6. HTAs should consider a wide range of evidence and outcomes. 7. HTAs should consider a full societal perspective. 8. HTAs should explicitly characterize uncertainty surrounding estimates. 9. HTAs should consider and address issues of generalizability and transferability. 10. HTAs should actively engage all stakeholder groups. 11. Those undertaking HTAs should actively seek all available data. 12. The implementation of HTA findings needs to be monitored. 13. HTAs should be timely but separate from other regulatory review. 14. HTA findings need to be communicated appropriately to different decision makers. 15. The link between HTA and decision making processes needs to be transparent and clearly defined."

Subsequently, the same group of scholars investigated the extent to which the proposed principles are supported and used by HTA organizations, including the PBAC. While this group observed substantial cross-national differences, they rated the PBAC largely positively. Major concerns related to principles 3 (scope, in terms of technologies considered) and 7 (consideration of a full societal perspective); whereas less substantial concerns were reported regarding principles 2, 6, 9, 10, 13, and 14 (Neumann et al., 2010).

International Standards II: "Accountability for reasonableness" (A4R)

An influential proposal for ethically defensible principles for priority setting in health care was made by Norman Daniels and James Sabin (1997, 2002). In the name of A4R, they argued for four conditions, "1. publicity (decisions and their rationales must be publicly accessible); 2. relevance (rationales must rest on evidence, reasons, and principles that all fair-minded parties can agree are relevant); 3. appeals (a mechanism for challenge and dispute resolution, including the opportunity for revising decisions in light of further evidence or arguments; 4. enforcement (either voluntary or public regulation of the process to ensure that the first three conditions are met)."

Some HTA agencies, including the National Institute for Health and Care Excellence (NICE) in the United Kingdom, have officially adopted the principles of A4R. Perhaps not surprisingly, even by NICE, not all A4R criteria have been fully met (cf. Schlander, 2007a, 2008). With regard to the PBAC, the first two criteria deserve special attention. As to 1., the "transparency" of decisions and their rationales (cf. the "Public Summary Documents" published by PBAC) leaves room for improvement, and as to 2., some key issues related to "rationales, evidence, reasons, and principles" will be addressed in the following.

International Standards III: Relevant experience in other jurisdictions

There is now a substantial literature comparing the results of PBAC evaluations with assessment and appraisal determinations in other jurisdictions (see, for example: Bae et al., 2015; Wonder and Chin, 2015; Woods et al., 2015; Spinner et al., 2013; Chim et al., 2010; Clement et al., 2009; Raftery, 2008; etc.). Conspicuous international differences notwithstanding, Ruth Lopert and David Henry (2002) noted that the problems arising from the intent to provide innovative medicines at an affordable cost are not unique to Australia. The observation encourages international comparisons, but it cannot exclude the possibility that certain parts of the challenges, that the PBAC and the PBS – like some other HTA agencies and collectively financed health schemes – are facing, might be endogenous.

Over-reliance on cost-utility analysis?

The new Guidelines (pp. 93ff.) strongly recommend cost-effectiveness (CEA) and/or – where possible – cost-utility analysis (CUA) as the preferred type of economic evaluation, which should be done from a "health care system" perspective and report its main results as incremental cost-effectiveness ratios (ICERs), for the proposed treatment compared with the comparator.[4] The Guidelines discourage alternative types of evaluation, explicitly mentioning cost-benefit (CBA) and cost-consequence analysis (CCA).[5]

The narrow focus on CEA and/or CUA with a preference for the latter raises concern in light of recent scientific developments (in the health economics discipline) and international experience (of HTA agencies), for a number of reasons[6] that include: 1. The reasoning offered in Appendix 6, section A6.1 (Appendices, pp. 20f.), rejecting CBA, appears overstated and partially misleading. 2. The implied superiority of the multi-attribute utility instruments (MAUIs) used to calculate preference-weights for the computation of QALYs (the new Guidelines mention the EQ-5D, SF-6D, AQoL, but not the Canadian HUI; pp. 57f.) is not clear; as the various MAUIs differ greatly in the structure and content of their descriptive systems, in their implicit measurement scales, in their sensitivity to physical and psychosocial dimensions of health, and thus in the health state valuations they generate (Richardson et al., 2015a,b). 3. ICERs, like all ratios, are "blind" to the size of the numerator and denominator and thus to the dimension of a program under consideration for funding, with far-reaching implications that may be highly problematic in decision-making contexts (Gafni and Birch, 1993, 2003, 2006). 4. Despite the need for a benchmark, it remains unclear what an appropriate cost-effectiveness threshold should be. 5. Importantly, the concept of a benchmark rests on the assumption that the objectives of Medicare and the PBS were to maximize the sum total of health gains of the population – which was found to be "descriptively flawed" (e.g., Dolan et al., 2005). 6. Even the (weaker) assumption that the social desirability of an intervention increases with decreasing cost per QALY gained cannot be generalized, as there are apparently dimensions of social value not captured by the QALY. Conventional QALYs reflect life expectancy and individual preferences for health states only. They exclude relevant social norms and preferences, such as (but not limited to) a strong and well-documented preference to prioritize health care for the worse off, for those in more urgent conditions, for the so called "rule of rescue", as well as preferences for equity including resource sharing per se, even if this is associated with efficiency losses (e.g., in Australia, Nord et al., 1995, and Richardson et al., 2012, 2015c; for recent reviews, see Nord and Johansen, 2014, and Schlander et al., 2014).

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[4] Technical note on comparator selection: In section 2 of the new Guidelines, the main comparator is (appropriately) defined as the therapy likely to be most replaced in practice; the required justification (new Guidelines, p. 16), however, creates a potential inconsistency by stipulating that "where multiple alternative therapies could be used for the majority of patients", the PBAC should refer to "the least expensive alternative medicine" – what should be done, if the alternative most used in practice and most likely to be replaced is not odentical with the cheapest alternative?

[5] The relatively less relevant case of cost-minimization analysis will not be discussed here.

[6] Technical note: less relevant in the present context and thus not discussed here is the fact that over-reliance on QALYs may contribute to the neglect of relevant clinical evidence, which cannot be converted easily into utility weights (Schlander, 2007b).

To complicate matters further, even the inclusion of social preferences into the evaluation paradigm cannot omit the need for a prior normative commitment, reflecting legal and institutional context (Roth, 2002); in contrast to rights and needs, individual preferences and tastes do not translate into moral duties of others.

Practical relevance for PBAC decision making

These are not merely theoretical considerations. They might explain, at least in part, cases of discordant PBAC recommendations and PBS / Government reimbursement decisions. For example:

In 2002 the Australian coalition government decided to reject a proposal by PBAC to list sildenafil, and simultaneously discontinued formulary listing of two other products (with alprostadil as the active ingredient) for erectile dysfunction (Kay Patterson, 2002).

Another example is the case of trastuzumab for the treatment of metastatic breast cancer overexpressing the HER-2 protein. A recommendation by PBAC to not list the anticancer drug was in effect overruled by the then-Minister for Health, Michael Wooldridge, in 2001. While the PBAC had found the drug not cost effective, the Health Minister decided to set up a special program to fund the drug independently from the PBS (MacKenzie et al., 2008; Doecke, 2011).

In yet another controversial case, the personal intervention by the then-Prime Minister, John Howard, led to a re-evaluation by PBAC of a new quadrivalent vaccine against human papilloma virus (HPV) infections, which offers effective prevention of certain types of cervical cancer in young women. The PBAC reacted by convening a special meeting and revoked its initially negative evaluation of the product, reportedly after the manufacturer had adjusted the price (Haas, 2007).

These examples illustrate the practical relevance of the mismatch between the logic of cost effectiveness – the predominant driver of PBAC recommendations – and prevalent social value judgments.

Implications for the new PBAC Guidelines

No doubt, the PBAC is aware that CUA has little to nothing to say about distributive justice and values such as solidarity, autonomy, and equity (cf. Whitty and Littlejohns, 2015). "Other factors" (beyond cost per QALY gained and uncertainty around its estimate) reportedly considered by PBAC include clinical need (especially in the absence of alternative treatment options) or clinical significance, the potential total cost to the PBS and/or Government health budgets, the potential for adverse population outcomes (e.g., the PBAC may restrict subsidized use of antibiotics for concern about the development of resistance), the affordability to the patient, and the "rule of rescue" (according to the Australian definition, reserved for rare, severe conditions with no alternative treatment), and more broadly – in some retrospective analyses – severity of disease (e.g., Harris et al., 2008; Clement et al., 2009; Lopert, 2009; Chim et al., 2010; Mauskopf et al., 2013).

However, with the exception of budget impact projections, the new Guidelines remain silent on any "other factors" that the PBAC may (or may not) consider in its appraisals. This gap is not conducive to a systematic consideration of social preferences for health care priority setting.

Past recommendations by PBAC appear to have been driven primarily by cost per life year gained or cost per QALY gained (cf., George et al., 2001; Harris et al., 2008; Chim et al., 2010). The new Guidelines reinforce and consolidate this focus on CUA, despite the problems and challenges arising from the implied narrow definition of value. There is an associated risk: In case social value judgments beyond the search for efficiency were not incorporated in the resource allocation decision process in a more systematic and transparent way, this might create potential opportunities for stakeholder groups with vested interests to launch media campaigns and make other attempts to interfere, appealing to public sentiments and the "rule of rescue." As was suggested earlier by MacKenzie and colleagues (2008), this might in turn contribute to arbitrary advantages for those resourceful enough to create

visibility and public attention, at the expense of less powerful and less vocal patient groups – in other words, undermine the authority of the PBAC and lead to injustice.

Conclusions and recommendations

The draft new Guidelines imply strengthening the focus of PBAC's processes on CUA, which would allow for a technically feasible, yet not necessarily always reliable, computation of ICERs as a measure of purported "efficiency." Within this context, the draft Guidelines reflect in many respects recent international developments. The context however implies reinforcing an efficiency paradigm up to the point of "changing the [social] problem to fit the solution" (Birch and Gafni, 1993), at a time, when other jurisdictions rejected this paradigm (e.g., Germany, USA) and when international HTA agencies created exemptions in order to escape from some of its most problematic consequences. For example, NICE in the United Kingdom relaxed the efficiency criterion for end-of-life treatments and inaugurated a separate Highly Specialised Technology (HST) evaluation process for interventions for ultra-orphan diseases, which does not apply cost per QALY calculations.

While implying increased opportunities for early consultation requirements, increased flexibility in terms of analytic approach would allow selecting the method most appropriate to solve the specific decision problem at hand. Methodological pluralism, as was advocated by the SwissHTA multi-stakeholder consensus (cf. Schlander et al., 2012), might be embedded in a multi-criteria decision making concept, that should identify the dimensions and sources of social value in a transparent way.

As a first step towards a more comprehensive and internally consistent framework ("social cost-value analysis", SCVA; cf. Richardson and McKie, 2007), it might be accompanied by a greater role of budget impact analysis, reflecting the opportunity cost of adopting a new program at the level of the PBS or Government health care budgets.

Moving forward in this direction, instead of backward orientation towards conventional CUA, might help resolving a number of problems that cannot resolved by, if not arise – at least in part – from deficiencies of, the logic of effectiveness:

Special Issues I: Orphan Medicinal Products (OMPs) or: "should we value rarity?"

Rare and ultra-rare disorders were broadly neglected ("orphans") prior to the introduction of a variety of economic incentives for manufacturers in many jurisdictions. The relatively high fixed cost, especially of pharmaceutical research and development, combined with low prevalence rates in many cases necessarily results in high drug acquisition costs per patient. Comparing social value and budget impact (opportunity cost) of (ultra-)rare disease programs might avoid either disenfranchising patients with rare and ultra-rare disorders from effective treatment options on grounds of cost-ineffectiveness or creating artificially defined exemptions (such as in the United Kingdom; cf. Tordrup et al., 2014).

Special issues II: Cancer and end-of-life treatments

Largely similar considerations apply to the evaluation of cancer treatments, in particular when the disease entity is rare. Sometimes, issues of clinical evidence add to the complexity, for example, when cross-over of control patients to the more effective treatment in pivotal trials is ethically required but precludes generation of long-term effectiveness data expected by HTA agencies.

Special issues III: Interventions for minor health problems

On the other hand, sildenafil, tattoo removals, and other treatments for relatively mild health problems would no longer be commendable for listing on grounds of cost-effectiveness, because a relatively high PBS / Government budget impact would be met by a relatively low social valuation of coverage. Affordability of treatment from the patient's perspective and minor impairments or self-limiting health problems only would be more relevant than cost-effectiveness.

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