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PBS Post-Market Review Team
Department of Health and Ageing
MDP 900
GOP Box 9848
CANBERRA ACT 2601
Via email to: PBSpostmarket@health.gov.au

14 September 2015

Dear Sir/Madam

Re: Public Consultation on the Pharmaceutical Benefits Advisory Committee (PBAC) Guidelines Review

Roche welcomes the Review of the Guidelines for Preparing Submissions to the Pharmaceutical Benefits Advisory Committee (PBAC) and the opportunity to comment via public consultation on the 'Items to be Included in the Review'. The Review is an opportunity to ensure the PBAC Guidelines are aligned with world best practice, incorporate new, updated and proven health technology assessment (HTA) methodologies and allow for consistency and transparency in how issues should be appropriately addressed.

Roche would like to provide comment on new issues and methodologies that it believes should be addressed within the PBAC Guidelines Review, as well as the importance of a review of evaluation processes alongside the review of methods. Both need to be considered in tandem if Australia is to be a benchmark for best practice health technology assessment.

Roche believes the reimbursement system for medicines in Australia needs to consider:

- Fit-for-purpose evaluation of medicines (taking into account budget impact, level of innovation and complexity, rarity of disease, unmet need and clinical benefit);
- Increased citizen, patient, clinician and academic involvement in decision-making, with improved transparency around decision-making and criteria;
- Incorporation of societal values and costs/benefits beyond the health system into the decision-making process; and
- Earlier and increased engagement between all stakeholders.

The importance of these factors, which represent standard practice in other developed countries using health technology assessment practices (e.g. the UK, Canada, and The Netherlands) was raised by numerous stakeholders during the Inquiry by the Senate Community Affairs References Committee on the *Availability of New, Innovative and Specialist Cancer Drugs in Australia*. Consequently, these factors need to be considered in the Review of the PBAC Methods Guidelines and the review of reimbursement processes.

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Review of PBAC Guidelines

Roche requests that the following five issues be addressed in the Guidelines Review in order to ensure:

- the assessments are based on scientifically sound and adequate methodologies and a broad concept of value, and they take into account all relevant parties and effects, as well as all available data and evidence;
- the scope of health technology assessments are fit-for-purpose; and
- there is increased objectivity and transparency in the methods used and decisions made.

1. Measuring and assessing the broad value of medicines

Australia currently applies a "one-size-fits-all" approach to assessing the value of medicines and a narrow conception of what aspects of value matter. Australia is lagging other developed countries that take a more flexible approach, including involving the broader community in considering the ethical and social components of value. The PBAC Guidelines need to provide methodological guidance as to how these important components of value can be considered in the reimbursement submission, assessment and decision-making process.

The current PBAC Guidelines focus on incremental cost-effectiveness ratios (which only consider some aspects of value, such as survival and quality of life), and budget impact. This frequently misses important value elements of medicines, including productivity gains, benefits and reduced burden for carers and the community, efficiency of healthcare delivery for healthcare professionals, improving medicine administration, improvements in patient compliance, incremental progress in disease management and the benefits of treatments in rare diseases where robust clinical trials are challenging to conduct. Not including these components of value presents equity issues for those patients who are unfortunate enough to be diagnosed with a rare disease or a disease where the value is measured in a non-standardised way, such as productivity benefits.

Roche recommends greater incorporation of societal values and benefits beyond the health system (such as productivity gains, social welfare and benefits to carers) into the PBAC Guidelines and decision-making process. At a time where Australia faces an ageing population and is focused on ensuring a productive and growing workforce, these elements must be given due consideration. While methodological challenges exist, ignoring productivity gains or losses, as well as benefits to carers and the social welfare system, is unlikely to be the right approach. Discrete choice experiments have been used in other jurisdictions, such as Germany, as a methodology for ensuring patient preferences are incorporated in a rigorous and robust way in the evaluation and assessment process for new medicines.

2. Fit-for-Purpose Assessments for Rare Diseases

Analysis contained in a report developed by The McKell Institute[1] has found that Australians are generally waiting from 2 to 4 years longer for access to rare disease therapies available in comparable countries like the UK, Canada, Germany and The Netherlands[2]. Some medications remain unavailable 8 years after becoming available overseas. Many rare disease therapies available overseas are unlikely to be available in Australia without policy reform and changes to the PBAC Guidelines to ensure a more fit-for-purpose evaluation when considering data requirements for rare diseases, where there is high unmet need and low budget impact.

A new approach is needed that considers the full scope of rare disease management, brings Australia closer to international standards on the definitions of rare diseases and evidence requirements for treatments, and adopts greater flexibility in the assessment of cost-effectiveness. The McKell report recommends a new approach to funding therapies for rare diseases based on Multi-Criteria Decision Analysis (MCDA), which would better align with Australia's National Medicines Policy and reflect international best practice.

Although this is an approach recommended for rare diseases, it could also be applied across all health conditions to assist with defining value more broadly.

The PBAC Guidelines need to acknowledge the difficulties associated with evidentiary requirements for rare diseases, where there is high unmet need, and consider the inclusion of an approach, such as MCDA, when assessing the broad value of medicines for rare diseases. MCDA incorporates numerous decision criteria and allows for transparent, weighted consideration of the criteria.

MCDA has been developed for a number of complex decision-making situations and applied to funding of new therapies for rare diseases internationally. MCDA has recently been adopted by the National Institute for Health and Care Excellence (NICE) in the UK in its decisions around funding ultra-orphan drugs, and represents international best practice. It employs the underlying philosophy of HTA assessment while reflecting the unique nature of therapies for rare diseases.

MCDA is a way of looking at complex decisions that are characterised by a mixture of monetary and non-monetary objectives, and of breaking decisions down into more manageable pieces. It would allow judgments to be made on individual criteria relevant to rare diseases such as rarity, disease severity, unmet need or degree of uncertainty around efficacy. While it is acknowledged that the PBAC takes non-monetary benefits into account, this is not formally weighted nor captured in the decision. Under MCDA these individual judgments may be weighted and jointly assessed to provide an overall picture for decision makers.

3. Evidentiary Requirements for Biosimilars

The PBAC Guidelines should include clear guidance on the evidentiary requirements for biosimilar medicines to determine appropriateness of switching and substitutability, and other matters to be considered by PBAC such as mode of administration, patient vs healthcare professional administration, delivery devices, need for patient education, potential for use in combination with other biologics etc. Roche agrees with the Medicines Australia submission that biosimilar medicines follow the major submission pathway, given the need for a thorough clinical evaluation of the available evidence to support switching and pharmacist substitution. This approach can be expected to enhance public confidence that decisions are evidence-based and appropriate.

4. Choice of Comparator

Roche is also concerned that the PBAC's approach to selecting medicine comparators for HTA is not consistent and may include treatments that are not appropriate, not registered for a particular relevant indication, nor supported by evidence. The PBAC Guidelines consider the appropriate comparator is the most commonly used medicine in Australian clinical practice. However, quality evidence may be limited for off-label use and listings, and comparisons outside of a registered label may not represent quality use of medicine. Clear guidance in the PBAC Guidelines on the appropriate comparator is needed to ensure consistent approaches are applied, and to enhance transparency of decision making in terms of choice of comparator.

5. Transparency of criteria for decision-making

It is requested that clear decision-making criteria be incorporated within the PBAC Guidelines to ensure that the factors considered in decision making are transparent and that they reflect the needs and values of the population. For example, in late 2015, Pharmac in New Zealand will be introducing new 'Factors for Consideration' in decision-making. These include: need; health benefits; costs and savings; and suitability of the health technology from the perspective of the patient, family and society (https://www.pharmac.health.nz/medicines/how-medicines-are-funded/factors-for-consideration/).

However, it is noted that no weighting will be applied to these factors, which does not fully address the need for transparency. MCDA provides this weighting and allows for transparency in decision-making.

Review of Reimbursement Processes

Alongside the Guidelines Review of methodologies, the planned review of reimbursement processes needs to ensure:

- the perspectives of all relevant stakeholders are taken into consideration in a timely way; and
- the decisions should reflect the needs and values of the Australian population.

It is important that the Australian community has a voice in determining what is value for money in HTA. These values can only be derived through a process that allows active participation by citizens and a clear set of decision-making principles reflecting society's preferences. These decision-making frameworks have been considered in other HTA countries. In the UK, patients and citizens are involved in setting decision-making criteria and participate on the HTA Committee. In Canada, patients participate on the HTA Committee and, as part of the pan-Canadian Oncology Drug Review, patient advocacy groups provide input at the beginning and throughout the evaluation process. This is a point of difference with the Australian system, where input from patient groups is only considered immediately prior to PBAC consideration in the form of Public Comments and PBAC hearings. Involvement of the community throughout the evaluation process is key to ensure a clear, consistent and effective process of valuing medicines more broadly.

The overall process of PBAC assessment could be made more fit-for-purpose, in line with the Government's commitment to reducing "red tape". In this respect, Roche agrees with the Medicines Australia submission that there is a need to establish a process for tiering of submissions. A more comprehensive evaluation is proposed for complex applications and innovative medicines that are often associated with higher budget impact and therapeutic value, while a less comprehensive evaluation is proposed for treatments associated with a low budget impact, treatments for rare diseases and those with a comparable clinical benefit and cost to existing therapies.

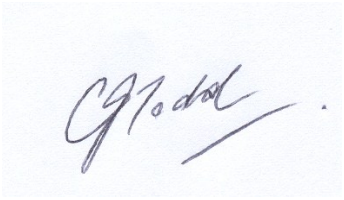
Roche also requests that the review of the reimbursement process incorporates a review of the co-dependent technology process. The uncertainties associated with co-dependent submission milestones and timelines make it very difficult to plan and ensure timely access of targeted therapies for patients.

Conclusion

In conclusion, the inclusion of well-documented, well-supported, clear and transparent methodologies that represent international best practice will increase the quality, consistency and alignment of health technology assessment in Australia. The use of robust approaches, consistency and alignment across stakeholders, in turn, has the potential to reduce resource requirements of the sponsor, evaluation units, the Department of Health and the PBAC in the assessment and appraisal of new medicines.

What is also important is that the use of world-class HTA methodology leads to appropriate decision-making that accounts for the full value that these medicines bring to Australian society.

Yours sincerely



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References/Notes

1. The McKell Institute (November 2014). Funding Rare Disease Therapies in Australia – Ensuring equitable access to health care for all Australians.
2. For example, alglucosidase alfa (Myozyme), a late-onset Pompe disease therapy, was listed in England, Netherlands and Germany in 2006 and was only PBS-listed in Australia for late-onset Pompe disease from September 2015.