Submission to the draft revised Pharmaceutical Benefits Advisory Committee Guidelines (PBAC) Public Consultation (draft version 5.0)

Prepared by the Cancer Drugs Alliance, April 2016

Executive Summary

The Cancer Drugs Alliance (CDA) welcomes the opportunity to provide comment on the draft revised Pharmaceutical Benefits Advisory Committee (PBAC) Guidelines (draft version 5.0).

The task of the PBAC is an important one and appropriately, the Government is now engaging in broad consultation with a range of stakeholder groups in recognition of the emerging trends around the world in the development of life-saving medicines. Australia urgently needs to adjust the framework for approval and reimbursement of such medicines so that patients with cancer can access innovative new drugs in a timely manner.

Cancer, including rare and less common cancers, presents a significant burden on society with 1 in 2 Australians expected to develop cancer in their lifetime and over 45,000 Australians each year dying from the disease, around 50% from rare and less common cancers.

Australian patients with cancer experience significant delays in accessing affordable new cancer medicines compared to patients in other parts of the developed world, a problem that is heightened for patients with rare and less common cancers (RLC cancers). To improve the current system in order to ensure more timely and affordable access to cancer medicines for Australian patients, the CDA recommends a number of practical changes to the PBAC Guidelines in five key areas:

1. Adopting international best practice in health technology assessment (HTA) that will position Australia as a world leader in providing earlier and affordable access to cancer medicines for patients.
2. Flexibility in considering evidentiary requirements for novel medicines
3. Provision for greater input from patients
4. Provision for greater input from clinicians.
5. Streamlined regulatory and reimbursement processes between the Therapeutic Goods Administration (TGA), the Medical Services Advisory Committee (MSAC) and the PBAC
6. The provision of clear guidelines regarding the type of real world evidence collected following a reimbursement decision that might inform a National Cancer Registry (a clinical quality registry).

Introduction

Cancer is a significant problem for society with 1 in 2 Australians expected to develop cancer in their lifetime and 1 in 5 dying from cancer before the age of 85 years. Cancer accounted for approximately 19 per cent of the total disease burden in 2012, greater than any other disease. Cancer kills 45,000 Australians each year accounting for 3 in 10 deaths, and around 22,000 of these deaths are due to RLC cancers.

Australian cancer patients face significant delays and expense in accessing new cancer drugs, or worse, never receive these medicines. For patients with RLC cancers in particular, missing out on the opportunity to receive potentially life-saving medication is often the norm.

In this submission, the CDA has put forward six recommendations that we believe will improve the operation of the PBS by making the assessment of new medicines under the PBAC Guidelines more patient-oriented and flexible.

Recommendations

Recommendation One – Adopting international best practice in health technology assessment (HTA)

The adoption of international best practices requires the PBAC Guidelines to make changes in the following areas:

a) Reduce reliance on cost-effectiveness
b) Adopting best practice from the USA and EU
c) Appropriate use of Managed Entry Schemes (MES)

Reduce reliance on cost-effectiveness

The current Guidelines are written so as to rely heavily on assessments of cost effectiveness. The CDA believes the PBAC needs to shift its focus away from a predominantly economic analysis and the perceived cost effectiveness of a drug, and instead base assessments primarily on the value of a drug, on the impacts that medicines can have on improving a patient's quality of life and survival as well as other key endpoints important to patients. Reliance on cost effectiveness assessments is already scientifically imperfect and problematic from the community perspective. New models that incorporate additional mechanisms to value drugs that improve patients' lives need to be explored, such as appropriate use of Managed Entry Schemes.

Adopting best practice from the USA and EU (including Germany, France, Spain, UK)

Other jurisdictions, including a number of countries in the European Union, have developed mechanisms that allow more timely access to new medicines, including cancer medicines. These counties include, but are not limited to Germany, France and Spain. The CDA believes that the PBAC Guidelines may be able to adapt many of these mechanisms to provide earlier access to cancer medicines to Australians.

Appropriate use of Managed Entry Schemes (MES)

Managed Entry Schemes (MES) have been adopted for a number of cancer medicines listed on the PBS over the past 2 years. Used appropriately, MES can help to overcome uncertainties potentially leading to more timely access to innovative new drugs. A carefully modelled MES may provide a basis for a win-win-win for Government, the pharmaceutical industry, and most importantly cancer patients who will be able to gain access to medicines sooner.
To enable more efficient data collection for MES, the CDA supports the creation of a National Cancer Registry (a clinical quality registry).

Where other countries have introduced reform based on MES, clear improvements in access to cancer medicines can been seen, both in terms of faster approval times and more medicines being available to patients.

Recommendation Two - Flexibility in considering evidentiary requirements for novel medicines

Recognising that to keep pace with the rapid technological advances in cancer treatment, a modern, adaptable system is required that is flexible. The current evidentiary requirements are rigid, often are out dated and may not reflect current clinical practice.

The CDA believes that PBAC should adopt a flexible approach to evidentiary standards and the assessment of innovative new therapies.

Recommendation Three - Provision for greater input from patients

This recommendation was partly informed by work undertaken by Kreab Research, which was focussed on consumer sentiment towards cancer drugs and treatments and how to resolve the issues identified by consumer input. Seventeen patient representatives from 15 consumer organisations took part in the survey. This research identified that consumer groups and patients felt largely left out of PBAC considerations, discouraging them from making submissions. Consumer stakeholder groups view Departmental policies and procedures in this area as "outdated, rigid and impenetrable".

Some key points included:

- A need for greater communication with consumers and consumer groups (including through social media);
- Improved guidance on how to make submissions, how submissions are used and how decisions are made;
- Having at least one other consumer representative on the PBAC with a cancer background; and
- Establishing a consumer sub-committee that the PBAC routinely consults with regarding specific conditions.

If the latter two suggestions were adopted, this consumer sub-committee could routinely be briefed by the Department, the relevant industry sponsor and appropriate clinicians in order to reach a carefully considered position on the value of each new medicine. This may require the consumer sub-committee to seek further community input as part of their deliberations.
It would be paramount that this input provides information to assist PBAC deliberations about the true value of a new medicine rather than cost-effectiveness *per se* and as such, a policy document that outlines how such input will truly inform decision-making would need to be developed and adopted by both the sector broadly as well as the Government.

Recommendation Four - Provision for greater input from clinicians

The CDA believes that there is value in earlier engagement of experts in the evaluation process. Early clinical and scientific input from clinicians who are experts in relevant fields could help address technical and clinical issues as well as important information on

treatment algorithms, comparators, and the presumed clinical value of a new medicine. Some of the important areas of clinical input may include:

- Level of unmet need;
- Incremental clinical benefit;
- Severity of disease; and
- Level of innovation (e.g. first-in-class/new mechanism of action)

While there is currently a process for individual expert clinician input into PBAC decision-making (usually at the invitation of the industry sponsor), often involving individuals attending formal PBAC meetings for very short times, how this input is adopted into decision-making (if at all) is not transparent. Alternatively, the process could be improved through the establishment of a forum for a robust discussion between the PBAC and expert clinicians, in which concerns about costs and cost-effectiveness raised by health economists versus the real value of a new medicine to patients can be debated. Providing accountable mechanisms as to how such input could be incorporated into decision-making would offer the community some confidence that the decision to recommend a drug or not has been taken in the knowledge of a broad understanding of the real value of such medicines.

Aside from the importance of early expert clinical input into the evaluation of individual drug submissions, there are a range of ways that the clinical community can be engaged to improve the current reimbursement system more broadly. Organisations such as the Medical Oncology Group of Australia (MOGA) and the Private Cancer Physicians Australia (PCPA) meet with the PBAC quarterly to review upcoming medicines. To increase their value to the process, several measures could be taken, including:

- To formalise the oncology and haematology panel from MOGA, PCPA & HSANZ for the purposes of horizon scanning. These specialist groups should also attend early stakeholder meetings. Oncology drugs would form the pilot, with other specialties being integrated as appropriate;
- Approaches as to how to deal with new therapies that are relevant to patients with RLC cancers could be considered at both a practical and policy level;
- These groups could consider/review data emerging from a National Cancer Registry (NCR);
- The agendas and minutes from these meetings should be published on the PBS website; and
- These panels could be tasked with creating a list of all new molecular entities

Recommendation Five - Streamlined regulatory and reimbursement processes between the Therapeutic Goods Administration (TGA), the Medical Services Advisory Committee and the PBAC

The PBAC Guidelines, while allowing for some coordination with TGA through the parallel processing provisions, need to be improved to allow greater streamlining. The PBAC Guidelines should consider the possibility of utilising provisions, such as MES, to narrow the gap between registration and reimbursement.

Recommendation Six - The provision of clear guidelines regarding what type of real world evidence collected following a reimbursement decision that might inform a national cancer quality registry

The establishment of a National Cancer Registry (a clinical quality registry) would provide real world data that would link to MES. Real world data would better support these MES to overcome uncertainties and improve access to innovative new drugs.

The establishment of a registry would also provide an extensive measure of appropriateness and effectiveness of cancer drugs and provide insight into the performance of innovative new therapies.

Conclusion

Through improvements to the PBAC process, we believe Australians can potentially lead the world in developing a sustainable, equitable and fit-for-purpose HTA system that will not only improve patient access to cancer medicines but all innovative medicines in the future. The CDA hopes this submission will provide useful suggestions to help finalise the current PBAC Guidelines Review.

Ultimately, a process that is modern, flexible and inclusive will be a positive development for all stakeholders. The Guidelines ought to involve patients and clinical experts at an earlier stage and move away from an over reliance on cost effectiveness and more considered evaluation of true clinical and social value. Most importantly, it is vital that the Guidelines continue to strive to make it easier for medical professionals to give Australians the treatment they need and deserve.

Appendix – About the Cancer Drugs Alliance

The Cancer Drugs Alliance (CDA) is a not-for-profit multi-stakeholder organisation committed to improving timely and affordable access to cancer medicines and achieving the best outcomes for Australian cancer patients. Membership of the CDA is comprised of practising oncologists, haematologists, representatives from cancer patient support and advocacy groups, and pharmaceutical companies currently providing cancer treatments to the Australian community.

The CDA aims to draw much-needed attention to the serious issue of inequitable, unaffordable and delayed access to cancer medicines in Australia, which is seeing many Australian cancer patients denied access to, or paying great sums in out-of-pocket expenses for, new cancer medicines that are readily available in other countries.

The CDA does not advocate for any one cancer treatment, it seeks to improve access for all Australian cancer patients and believes that only by bringing together the expertise of those engaged in cancer care, treatment and support will Australia achieve the shared goal of delivering world's best practice in cancer care and treatment.

For more information about the CDA please see our website at: www.cancerdrugsalliance.org.au

Alternatively please contact the CDA via email: info@cancerdrugsalliance.org.au