Submission to the Pharmaceutical Benefits Advisory Committee (PBAC) Guidelines Review

Prepared by the Cancer Drugs Alliance, September 2015

Executive Summary

The Cancer Drugs Alliance (CDA) welcomes the opportunity to provide comment on the Pharmaceutical Benefits Advisory Committee (PBAC) Guidelines Review. 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 of such medicines so that patients with cancer can access innovative new drugs so desperately required to improve their quality of life and survival in a timely manner.

Cancer, in particular rare and less common cancers, present 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 Pharmaceutical Benefits Scheme (PBS)-listed cancer medicines for Australian patients, the CDA recommends a number of practical changes to the PBAC Guidelines in four key areas:

1. earlier and greater involvement of patients and patient groups;
2. earlier and greater involvement of expert clinicians;
3. reducing the reliance on cost effectiveness in PBAC decisions, not only because such measures do not assess the true value of new medications but also because in many situations there is sufficient uncertainty about outcomes that calculations of cost-effectiveness inherently disadvantage patients by delaying access to novel drugs; and
4. tailoring the Guidelines to allow better coordination between TGA and PBAC processes to minimise the gap between registration and reimbursement, creating a system which facilitates immediate access to innovative new drugs at the time of TGA registration.

If the Guidelines address these four areas then it is our belief they will be more flexible, dynamic and responsive to patient needs.

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 accounts for approximately 19 per cent of the total disease burden in 2012,[1] 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.

1 Australian Institute of Health and Welfare & Australasian Association of Cancer Registries 2012. Cancer in Australia: an overview, 2012. Cancer series no. 74. Cat. no. CAN 70. Canberra: AIHW.

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.

Cancer patients unfortunately do not have the luxury of time to wait months or in some cases years for the best medicines to become available; all Australian patients need, expect and deserve timely and affordable access to available cancer medicines.

In this submission, the CDA has put forward 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.

The Current PBAC Guidelines need to change to allow Australia to keep pace with rapid advances in the management of cancer

The current PBAC Guidelines, while serving as a reasonably useful basis for the consideration and assessment of the previous generation of cytotoxic chemotherapy agents, need to be amended to address the rapid worldwide advances in the diagnosis and treatment of cancer. Just as knowledge of the innumerable sub-types of cancer, and understanding of the biology and natural history of cancer has changed so dramatically in the past decade, so have community and clinical expectations of what represents optimal care.

Evolving the PBAC Guidelines - Recommendations

To ensure that the PBAC Guidelines keep pace with the changing cancer landscape and the many innovative cancer medicines in development, the CDA has identified four areas for reform:

1. earlier and greater involvement of patients and patient groups;
2. earlier and greater involvement of expert clinicians;
3. reducing the reliance on cost effectiveness in PBAC decisions, not only because such measures do not assess the true value of new medications but also because in many situations there is sufficient uncertainty about outcomes that calculations of cost-effectiveness inherently disadvantage patients by delaying access to novel drugs; and
4. tailoring the Guidelines to allow better coordination between TGA, MSAC and PBAC processes to minimise the gap between registration and reimbursement, creating a system which facilitates immediate access to innovative new drugs at the time of TGA registration.

Recommendation 1 - Earlier and greater involvement of patients and patient groups

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. Our 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 2 - Earlier and greater involvement of expert 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 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 Chemotherapy 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 due in the next one-to-three years, prioritizing them based on their clinical value.

Recommendation 3 - Reducing the reliance on cost-effectiveness in PBAC decisions

The current Guidelines are written so as to rely heavily on assessments of cost effectiveness. Recognising that to keep pace with the rapid technological advances in cancer treatment, a modern, adaptable system is required that can provide affordable and timely access to new medicines for cancer patients. The CDA believes the PBAC needs to shift its focus away from strictly economic analysis and the perceived cost effectiveness of a drug, and instead base assessments primarily 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; that is, the true value of medicines. 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. For example, considerations of cost effectiveness need not impede considerations of livelihood, wellbeing and additional economic benefits that come with a consumer returning to the workforce and contributing to society, for example.

Recommendation 4 - Tailoring the Guidelines to allow better coordination between TGA, MSAC and PBAC processes

The PBAC Guidelines, while allowing for some coordination with TGA through the parallel processing provisions, need to be improved to allow greater streamlining to minimise the gap in access between registration and reimbursement. Indeed the decision that a medication should be reimbursed is often followed by many protracted months before patients can actually access such drugs (only in small part due to the need for Federal Cabinet approval for some drugs). Furthermore the linking of PBAC and MSAC processes whilst apparently logical conceptually, has only served to increase the delays by which patients can access the medications they need.

The CDA supports the creation of a National Chemotherapy Registry (NCR) that can leverage the concept of a managed entry scheme (MES) to a system that provides immediate access to cancer medicines at the time of TGA approval with real-world data providing the level of certainty to justify or negate the business case used to support a recommendation.

We urge the Government to consider the establishment of a carefully modelled 'interim access scheme'" that is a win-win for both Government and the pharmaceutical industry, and ensures the patient is not the 'meat in the sandwich' and can access medicines at the time of TGA approval.

Our specific suggestion is that an interim access program allowing immediate patient access from the time of TGA approval is piloted for a limited group of truly innovative medications in the first instance.

Where other countries have introduced reform, clear improvements in access to cancer medicines can been seen, both in terms of faster approval times and more medicines being available to patients. Through improvements to the PBAC process, we believe Australians can potentially lead the world in developing a sustainable, equitable and fit-for-purpose access system that will not only improve patient access to cancer medicines but all innovative medicines in the future.

Conclusion

The CDA hopes this submission will provide useful suggestions to reform the current PBAC Guidelines. Ultimately, a set of Guidelines 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 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.

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