Submission to the Expedited pathways for prescription medicines: Eligibility criteria and designation process
Prepared by the Cancer Drugs Alliance, December 2016

Executive Summary

The Cancer Drugs Alliance (CDA) welcomes the opportunity to provide comment on the consultation paper Expedited pathways for prescription medicines: Eligibility criteria and designation process.

The Therapeutic Goods Administration (TGA) plays an important role in ensuring the health and wellbeing of all Australians by providing effective and timely regulation of therapeutic goods. The Government should be commended for the broad consultation process it is undertaking to improve access to prescription medicines.

The CDA strongly believes that cancer medicines should be made available as soon as possible to patients and that the TGA can play an important role in achieving this goal.

The CDA supports the TGA adopting best practice models with accredited international partners such as the US Food and Drug Administration (FDA) and the European Medicines Agency (EMA).

Further, the CDA believes important new medicines which have already been approved by accredited partner agencies should be eligible for expedited or provisional approval.

Lastly, the CDA believes that when parallel applications (e.g. to TGA and EMA simultaneously) are being processed, mechanisms to expedite approval for important new drugs by sharing the workload required to review the chemistry, formulation and manufacturing of non-chemical entities (NCEs) as well as adverse events should be introduced in order to reduce the time for review.

A detailed list of our recommendations in response to the consultation paper is provided in the brief document below.

Moreover, we believe that a face-to-face workshop to consider the breadth of likely responses to this consultation process is warranted to maximise this unique opportunity to reform the means by which we regulate drugs in Australia in order to provide safe and effective pharmaceuticals to the community in the timeliest manner.

We would be pleased to have the opportunity to meet with TGA representatives to discuss these in more detail.

Introduction

Cancer, including rare and less common cancers (RLC), presents a significant burden on society with:

- 1 in 2 Australians expected to develop cancer in their lifetime
- 1 in 5 dying from cancer before the age of 85 years
- over 45,000 Australians dying each year from the disease (accounting for 3 in every 10 deaths), including around 50 per cent from rare and less common cancers.
- Cancer accounting for approximately 19 per cent of the total disease burden in 2012, greater than any other disease

Australian cancer patients face significant delays and expense in accessing new cancer drugs, or worse, never receive these medicines, compared to patients in other parts of the developed world.

For patients with RLC cancers in particular, missing out on the opportunity to receive potentially life-saving medication is often the norm, as there is often much more limited data available to support regulatory applications to treat such conditions.

The Expedited Pathways program is a very important change designed to improve access to important new drugs. While the CDA makes a number of suggestions for improving the program, this idea and the broad consultation that the government is undertaking should be commended.

Recommendations

Eligibility criteria

- Do the proposed criteria for Priority Review and Provisional Approval address the objectives of the expedited pathways?
The CDA broadly agrees with the classifications of ‘Priority Review’ and ‘Provisional Approval’ for expedited pathways. The proposed criteria included in the consultation paper (unmet need and serious illness) is supported by the CDA as the criteria for these new classifications.

- **What other considerations may need to be included?**

For Priority Review, the TGA needs to consider the use of data from accredited overseas agencies like the FDA and EMA to speed up approval for important new drugs. While we appreciate that conclusions from international review of submitted data may not always be available at the time of submission to the TGA due to parallel submissions, we suggest that there may still be opportunities for the TGA to consider. For example, is there a need for both the TGA and EMA to both review preclinical toxicology, chemistry and formulation? Couldn’t some of these tasks be shared (indeed could safety and potentially efficacy review be shared as well, especially if some of this information from a partner agency becomes available during the time that the TGA Review is still underway)?

For both Priority and Provisional Approval, where an accredited regulatory agency has already approved a drug, even for another indication, the CDA believes the TGA should have a specific set of metrics recognising the need to fast-track approval, as there is no need to analyse data around the preclinical toxicology, chemistry and formulation of the drug separately.

Similarly, where Priority or Provisional Approval is requested for a drug already approved by the TGA, a shorter time period should be required to expedite such approval should provisional approval for a new indication be requested.

**Designation process**

- **Is the proposed process and timing of the designation steps appropriate?**

The CDA broadly agrees with the proposed process and timing of the designation steps, with three important differences:

- Where review has been completed/undertaken by an accredited overseas agency, a shorter timeline for review and approval should apply, as there is no need to analyse as much data.
- For drugs that have already been approved for another indication, there should be a mechanism for expedited review and approval of these drugs, as there is no need to go through the full process of approval again.
- The CDA believes the timing of Priority Review designation should be 150 calendar days, not 150 working days. There should also be clear, transparent instructions and information about ‘stop the clock’ provisions in the timeframe, in order to give certainty to drug manufacturers, clinicians and consumers.

- **What other considerations may need to be taken into account in implementing the proposed designation process?**

Like the US FDA, the TGA should develop criteria and capabilities to offer ‘Breakthrough’ and ‘Accelerated’ status for important new drugs. The CDA accepts that the TGA does not have these capabilities at present. However, over the course of the first year of the new expedited pathways program, a scoping study should be undertaken to investigate this option.

**Duration of designation**

- **Should there be a three-month limit on the duration of the designation for Priority Review and Provisional Approval? If not, please provide reasons and suggest what could be an alternative time period.**

Where it is safe to do so, the CDA supports a 12-month duration for ‘Priority Review’ designation, and a 24-month duration on a Provisional Approval designation. These durations should be also subject to roll-over provisions, where appropriate. These longer durations are required as often, data takes a long time to collect through clinical trials, and three-months is far too short. Obviously, if there are bona-fide concerns with the safety of a drug, then the TGA should have the power to reverse a designation.

The diagram (Figure 1 in the consultation paper), does not adequately illustrate these timeframes and should be updated to detail specific timeframes at each stage of the process.

**Publication of TGA decisions**

- **Should we publish the outcomes of applications for Priority Review and/or Provisional Approval designation?**
The CDA believes in maximum transparency and as a result believes the TGA should publish the outcomes of all applications (including the date of submission, stop the clock provisions with some explanation and dates of approval).

- Should publication of both ‘eligible’ and ‘ineligible’ designation decisions occur?

The CDA believes in maximum transparency and as a result believes the TGA should publish the outcomes of applications, including both eligible and ineligible decisions.

- Should we publish whether a medicine has been registered through one of the expedited pathways?

The CDA believes in maximum transparency and as a result believes the TGA should publish medicines registered through the expedited pathways.

- If so, how much detail should be published and when should TGA decisions be published?

The CDA believes that applications, and the TGA’s decision on those applications should be published in real-time to ensure maximum transparency for involved parties. In addition to the date of approval, the CDA also supports publishing the date of submission.

Other considerations

- What other key issues should be considered in developing the Priority Review and Provisional Approval pathways?

  - The TGA should work with Medicines Australia (MA) to give consideration to ways of ensuring patients and clinicians are made aware of all company-initiated “Patient Familiarisation Programs (PFPs)”.
  - The TGA should also consider the impact of expedited pathways on the PBS. Where a pharmaceutical company undertakes a parallel submission to the TGA and PBS, and that drug is eligible for consideration under the expedited pathways program, the PBAC should endeavour to keep up with the TGA’s expedited pathways timeline.
  - The TGA should develop processes that do not disadvantage applications for drugs that treat RLC cancers, treatments which are sometimes associated with less safety and efficacy data.
  - To ensure community support, the TGA should include consumer and clinician stakeholder reference groups in the decision-making process when deciding the eligibility for expedited pathways. This could occur before a submission is formally submitted, with feedback included as part of the formal-decision making process. We see no reason why such consultation should delay an expedited pathway process.
  - The CDA strongly believes that post-marketing analysis of drug safety and efficacy for drugs approved under the Provisional pathway in particular, should be required in most, if not all instances. These should be modelled under the clinical quality registry model defined by the Australian Commission for Safety and Quality in Health Care.

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 visit www.cancerdrugsalliance.org.au or email: info@cancerdrugsalliance.org.au