CANCER
DRUGS
ALLIANCE

Submission to the Post-market Review of the Life Saving Drugs Programme Public Consultation

Prepared by the Cancer Drugs Alliance, November 2014

Introduction

Cancer is an area of high clinical need – it is not one disease, it is many hundred diseases and each of these can manifest differently in each cancer patient. Cancer is the leading cause of the total burden of disease and injury in Australia, accounting for approximately 19 per cent of the total disease burden in 2012.[1] One in two Australians will develop cancer in their lifetime and one in five will die from cancer before the age of 85 years.

Overall, cancer kills 45,000 Australians each year accounting for three in ten deaths; and around 22,000 of these deaths are due to rare and less common cancers, which account for seven per cent of the total burden of disease in Australia.

'Rare cancers' are defined as those with an incidence of less than six per 100,000 Australians per annum and 'less common' cancers as those with an incidence of between six and 12 (inclusive) per 100,000 Australians per annum[2].

Cancer represents an extremely complex challenge for our healthcare system. However, with advances in medical research and our understanding of the individuality of the disease, we are now increasingly able to define cancers by their genetic abnormalities through molecular diagnosis, target specific medicines to these genetic abnormalities and thereby increase the benefit of treatment.

Even amongst the commonest cancers there are very small numbers of patients with rare genetic abnormalities. For example, in lung cancer (10,193 total cases per annum in Australia[3]), 1% of non-small cell lung cancer patients have a mutation in a particular gene (known as ROS-1) which renders these tumours (cancers) more sensitive, than other common lung tumours, to a specific drug available in over 70 countries around the world and approved by the TGA in Australia, but not available to these patients.

Access to Medicines in Australia

Although the Pharmaceutical Benefits Scheme (PBS) has served Australians well for over 60 years, it has not kept pace with technological advances and complexity in cancer treatment and clinical practice. This means many Australian cancer patients face significant delays and expense in accessing new cancer drugs, or worse they are missing out altogether.

However, more specifically with respect to the rare cancer sub-types, the current guidelines used by the TGA and PBS significantly disadvantage these Australians requiring life-saving drugs.

Neither the TGA nor the PBS is designed or constituted to respond quickly to advances in medical treatment, particularly in cancer where treatments are becoming more personalised for an

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[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.

[2] Gatta et al., Rare Cancers are not so rare: The rare cancer burden in Europe. *European Journal of Cancer* 47, 2493-2511 (2011).

[3] 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.

increasingly smaller number of patients – i.e. as specific molecular or genetic changes define very small groups of tumours, it is increasingly difficult to demonstrate cost-effectiveness for life-saving drugs or indeed to conduct clinical trials that satisfy the normal regulatory processes. In fact in some cases, randomised trials (even conducted on a world-wide basis) are simply not possible.

Hence allowing these patients access to important anticancer dugs through the life-saving drugs program, hitherto not possible under the criteria used by the Department of Health is absolutely critical for these patients.

Even when there is evidence suggesting that a drug may be highly effective for a rare mutation, the extremely small patient numbers may mean that the data required to satisfy regulatory requirements cannot be generated; therefore TGA registration is not possible and hence PBAC submission prohibited. As a result, patients are missing out on cancer drugs that are considered very effective by their treating oncologists but cannot be accessed though the PBS or the Life Saving Drugs Programme (LSDP).

To clarify the lung cancer example referred to above, there are a total of 10,193 new patients diagnosed with lung cancer per annum in Australia[4]; approximately 1% of non-small cell lung cancer patients have a ROS-1 mutation. While there is evidence that suggests that crizotinib (Xalkori) may be highly effective for non-small cell lung cancer patients (NSCLC) with ROS-1 mutation, the small patient numbers means that the data required to satisfy regulatory requirements cannot be generated.

Another example that illustrates the issues with subgroups of patients is stomach cancer (2,015 total cases per annum in Australia5). One in 10 patients (i.e. approximately 200 new patients/year) have increased levels of a growth factor receptor on the surface of cells known as HER2. This same growth factor receptor is present in a small percentage of breast cancers and when present, patients derive substantial benefit from the drug trastuzumab (Herceptin). There is very good evidence to suggest that trastuzumab can significantly help patients with advanced, HER2-positive stomach cancer, although at this time it is only listed on the PBS for the treatment of patients with HER-2 positive breast cancer.

LSDP Review Opportunities

The LSDP provides subsidised access, for eligible patients, to life saving drugs for rare life-threatening conditions. This Review provides an opportunity to assess the current LSDP in order to ensure that Australians with the full range of rare conditions, including rare and less common cancers, have subsidised access to much-needed life-extending medicines.

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.

We must ensure that the system keeps pace with medical advances so that Australian patients with rare conditions can access new treatments when they become available. The CDA is therefore calling on the Government to allow for the LSDP to be revised to provide access to these drugs for patients with rare and less common cancers.

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[4] 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.

[5] 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.

The current LSDP requires a drug be registered by the TGA in order for that drug to be funded, but due to the small patient numbers, the level of available data often does not exist to meet the TGA regulatory requirements. The LSDP must therefore be amended to allow access to the appropriate drugs for these small populations of patients with rare or less common cancer, as recommended by the treating oncologist. It is also recommended that provisions be made to capture the real-world data through the development of a Registry that would measure outcomes (both benefits and serious side-effects) from the treatment of these patients as it becomes available through the LSDP.

The CDA recommends that the following change be made to the criteria by which the TGA and Pharmaceutical Benefits Advisory Committee provides affordable and ready access to a drug through the Life Saving Drugs Program:

A) The drug must be found to meet one of the following criteria:

> 1. There is a rare but clinically definable disease for which the drug is regarded as a proven therapeutic modality, i.e. approved for any related indication in cancer patients by the Therapeutic Goods Administration, or some other internationally agreed regulatory agency.
> **OR**
> 1b. There is a rare but clinically definable disease for which there is evidence that a drug may be highly effective, but for which insufficient patient numbers exist to satisfy the Therapeutic Goods Administration’s registration requirements, and a treating oncologist has recommended its usage based on existing international experience or data.

B) The patient and their clinician should agree to provide an agreed and predefined minimum dataset to assist in the collection of outcome data

C) Consideration be given to extending the definition of the LSDP to use for patients with cancers (or cancer subgroups) with an annual incidence of <12/100,000 Australians.

Conclusion

With advances in medicine and our understanding of the biology of cancer there are a growing number of cancers that we know to be rare or have rare definable subgroups (often based on genetic mutations), but for which the normal regulatory pathway is unsuitable, due to the small patient numbers.

Despite existing data suggesting there are drugs that are very likely to be highly effective for, these small patient numbers, the data required to satisfy regulatory requirements cannot be generated; therefore TGA registration is not possible. As a result, in many cases, patients are missing out on cancer drugs that are considered very effective by their treating oncologists because patients cannot access them though the PBS or the LSDP.

The CDA therefore calls on the Government to amend the LSDP to ensure that where evidence on the efficacy of a drug is limited, due to small patient numbers, that the drug be made available via the LSDP to the patient based on the recommendation of their treating oncologist.

Resolving the issues of timely and affordable access to cancer medicines for Australian patients is now critical and requires a long-term solution to cancer drug funding that is fair, equitable and sustainable.

Without significant long-term, sustainable reform, Australians will wait longer and longer to access to cancer medicines that are available elsewhere in the world.

The Cancer Drugs Alliance further calls on the Government to review the current funding processes for cancer medications and is seeking to work with all stakeholders to explore additional long-term and short-term, sustainable strategies to urgently address the crisis in the access to cancer medicines we are seeing in Australia.

The CDA welcomes the opportunity to provide this response to the Post-market Review of the Life Saving Drugs Programme Public Consultation. Australian patients can no longer afford to delay, we must act on their behalf and we must act quickly.

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

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