CanTeen National Office
GPO Box 3821
Sydney NSW 2001

Community Affairs References Committee
Parliament House
PO Box 6100,
Canberra ACT 2600
Email: community.affairs.sen@aph.gov.au
http://www.aph.gov.au/senate/committee/wit_sub/index.htm.

Dear Community Affairs References Committee,

Thank you for the opportunity to provide input to the Senate Inquiry into the availability of new, innovative and specialist cancer drugs in Australia. It is with great pleasure that I submit our response, prepared on behalf of CanTeen with the support of senior Youth Cancer Service representatives and other senior health stakeholders.

As part of the submission process I would be delighted if we could have the opportunity to appear before the Committee at a public hearing to further elaborate on the issues presented in our submission.

Yours sincerely,

Peter Orchard
CEO

Senate Inquiry into the availability of new, innovative and specialist cancer drugs in Australia

On 3 December 2014, the Senate referred the matter of new innovative and specialist cancer drugs in Australia to the Community Affairs References Committee for inquiry and report by 26 March 2015. Submissions are due 27 February 2015.

The terms of reference for the Senate Inquiry are the availability of new, innovative and specialist cancer drugs in Australia, with particular reference to:

(a) the timing and affordability of access for patients;
(b) the operation of the Pharmaceutical Benefits Advisory Committee and the Pharmaceutical Benefits Scheme in relation to such drugs, including the impact of delays in the approvals process for Australian patients;
(c) the impact on the quality of care available to cancer patients; and
(d) any related matters.

Background

The increasing demand for health services and increasing costs associated with new technologies and personalised medicine present a serious challenge for the Australian health system.[1,2]

Specific issues to Adolescents and Young Adults (AYA)

Young Australians face exceptionally difficult cancer journeys. The number of young people aged 15-24 years diagnosed with cancer each year is 1.5 times the number of children aged 0-14 years that are diagnosed.[3] Young people have significantly poorer survival rates than children and older adults in some of the cancers common in this age group.[4] Many of the cancers that affect young people are rare. Young people also present with a larger array of cancer types compared to older adults: 90% of the cancer burden is accounted for by 20 different cancer types.[5] Furthermore, young people also tend to present with cancer at a more advanced stage due to longer delays before diagnosis[6] and suffer higher rates of inferior psychosocial outcomes compared to other age groups.[7] This in turn, is associated with a poorer prognosis and a heightened risk to survival.[8] Consequently, for some cancers, young people show a much poorer response with the same treatments given to older adults or younger children.[9]

In order to rectify this situation, there is a pressing need for AYAs to be able to access new and innovative specialist cancer drugs. However, AYAs with cancer experience disproportionate difficulty in accessing such agents. This is largely due to factors preventing the clinical trials that are necessary prior to regulatory approval of new agents for AYA cancers, and issues of affordability, as summarised below.

There is a lack of clinical trials for the cancer subtypes commonly seen in AYAs

AYAs have a different spectrum of cancers to older adults, with even the most common subtypes of cancer being relatively rare. Because pharmaceutical companies do not usually devote significant research effort to rare diseases, no clinical trials are available for most of the cancer subtypes seen in AYAs.

A number of drugs developed for more common adult cancers also have activity in the types of cancer seen in AYAs. For instance, ruxolitinib, which was developed for older adults with myeloproliferative disease, has recently been reported to be effective in some AYAs with Philadelphia-like acute lymphoblastic leukaemia. Even though this drug appears effective in this disease, the PBS is unlikely to approve the indication until rigorous studies have been

undertaken. However, there is currently no incentive for pharmaceutical companies to undertake the clinical trials that are necessary to obtain an indication for this comparatively small market. As such, AYAs with cancer are unable to access potentially life-saving agents despite these agents being approved for other indications.

AYAs often cannot access clinical trials due to their age

Within the AYA age group (15 – 25 years), there is discrepancy of access to clinical trials. Most industry-sponsored studies stipulate a lower age limit of 18 years in the inclusion criteria, thereby precluding many younger adolescent patients from participating in the trial. There is usually no valid biological justification for this age-eligibility criteria, and various AYA cancer advocacy groups internationally have criticised this practice.

In the US and Europe, legislation has been passed encouraging pharmaceutical companies to develop drugs with paediatric indications (eg Best Pharmaceuticals for Children Act, 2002; Paediatric Research Equity Act, 2003; European Paediatric Medicine Regulation, 2007). In Australia, there is no equivalent legislation. Consequently, there is no incentive for pharmaceutical companies to seek the PBS listing of indications relating to cancers in children and adolescents.

Australian participation in international collaborative group clinical trials is becoming more restricted due to financial issues and regulatory requirements

Historically some younger adolescents have been able to access new agents via Australian paediatric oncology centres' participation in US Children's Oncology Group (COG) studies. However, in recent years the US Cancer Therapy Evaluation Program (CTEP), which determines whether non-US COG sites will have access to investigational new drugs, has not approved Australian sites for a number of new agents.

AYAs often cannot access collaborative group clinical trials due to their location of care

Despite the restrictions imposed by CTEP described above, a number of children and younger adolescents who are treated in Australian *paediatric* oncology centres have been able to access some new drugs via Australian participation in COG studies. While many COG studies have an upper age limit of at least 30 years to encourage AYA participation, most Australian young adults with cancer are treated in *adult* hospitals that are not able to access these studies due to COG's strict membership requirements.

AYAs often cannot access new drugs due to their high cost

If a new drug is approved and subsidised by the PBS for a specific indication, it is also theoretically available commercially "off licence" for other indications but at full price. New drugs are frequently extremely expensive and most AYAs and their families cannot afford them. While clinicians can request that their hospital pay for such drugs via individual patient usage applications, most public hospitals also cannot afford such drugs. Moreover, while one hospital may approve an individual patient usage application for a specific agent, another hospital may not, thereby creating further inequity of access.

To summarise, lack of access to new and innovative drugs will continue to exacerbate the lack of improvement seen in AYA cancer. Survival deficits in AYAs with cancer is associated with a relative lack of participation in clinical trials.[10] It is anticipated that facilitating access to clinical trials will improve AYAs' patient experiences, outcomes and ultimately survival in the future.[10,11] We believe that there is a pressing need to focus on the clinical trials that are required prior to product approval, as well as affordability of new drugs.

In preparing these recommendations, CanTeen has consulted with senior representatives from our Youth Cancer Services governance groups and other senior health stakeholders, summarised at Appendix 1.

CANTEEN RECOMMENDATIONS TO THE COMMITTEE:

1. A commitment to equitable, timely and affordable access to new cancer drugs, including:
   a) Greater financial support of clinical trial activity in hospitals treating adolescents and young adults with cancer.
   b) Establishing legislative incentives for pharmaceutical companies to develop paediatric and adolescent indications for new drugs.
   c) Encouraging pharmaceutical companies to change the lower age-eligibility criteria in clinical trials from 18-years to 15-years.
   d) Conditional drug approval by the TGA and PBAC for new drugs which show promise in patients with a rare disease, where the threshold for benefit under normal circumstances is impossible by virtue of rarity, and where there is clearly an unmet need. The definitions for these (rarity, burden of evidence, and unmet need) would come from expert advisory groups where a quorum of clinicians believes there is an unmet need and the drug has value. One model could include conditional access to new drugs with a sunset clause, subject to data collection, and through a limited number of high volume institutions. This managed entry scheme could be based on surrogate endpoints (such as efficacy in the adult population or in rare cancers disease free survival) with prior agreement that funding could be altered or withdrawn if the drug did not meet its efficacy targets (e.g. overall survival or efficacy in specific AYA studies). By increasing access to these cancer treatments, the relative disadvantage currently experienced by AYAs would be reduced.
   e) Addressing the needs of vulnerable cancer patients, including those living with rare cancers and residing in regional and remote areas of Australia.

2. There is a need for more active involvement of patients and consumer organisations in the PBAC decision-making process, to ensure that consumer needs are both understood and incorporated into the process of determining what treatments should be funded.

3. There is a need for greater transparency of the PBAC drug pricing policy, with particular reference to utilising the evidence to increase alignment between drug price and effectiveness.

4. There is a need for the PBAC to improve their ability to respond to the changing environment and increasing complexity of new cancer treatments.

5. Streamlining the burdensome research governance requirements that currently make it so time-consuming and difficult for clinicians to open clinical trials in their institutions, as this ultimately limits companies' ability to seek listing for cancer types that occur in AYAs.

References

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Appendix 1: Youth Cancer Services Governance Groups

In preparing this summary CanTeen has consulted with senior representatives from our Youth Cancer Services (YCS) governance groups, including YCS Leadership Group – Medical team, YCS Research Advisory Group, YCS Strategic Advisory Group and the CanTeen Board.

YCS Leadership Group – Medicos

Dr Michael Osborn*
Dr Antoinette Anazodo*
Dr Po-Ling Inglis*
Dr Lisa Orme*
Dr Rachel Hughes*

YCS Research Advisory Group

Professor David Currow (Chair)*
Dr Antoinette Anazodo*
Dr Cleola Anderiesz
Professor Afaf Girgis
A/Professor Stephan Jan
Professor Bogda Koczwara
Dr Wayne Nicholls
Dr Michael Osborn*
Dr David Thomas*
Dr Tim Threlfall
Professor Kate White

YCS Strategic Advisory Group

Dr Jenny Bartlett (Chair)*
Dr Antoinette Anazodo*
Dr Heather Buchan
Ms Noelle Cridland
Professor Marion Haas
Ms Caroline Nehill
Professor Ian Olver*
Ms Jenni Seton
Ms Kate Thompson
Professor Andrew Wilson*
Mr John de Zwart

** Representatives who were consulted as part of this submission.*