Brief

Business Review and Reporting Section
Prescription Medicines Authorisation Branch
Therapeutic Goods Administration
PO Box 100
WODEN ACT 2606

Date: 2nd December 2016.

Dear Business Review and Reporting Section, Prescription Medicines Authorisation Branch,

RE: Consultation on the orphan drug program, 2016 orphan drug program proposal, Version 1.1, October 2016

CanTeen is providing comments on the consultation items as requested by the TGA.

In summary, CanTeen would like the orphan drug program to consider the adolescent and young adult population as a unique group with a unique biology, different to that of paediatric or adult populations. This group is also disproportionately highly affected by rare cancers, and lack access to clinical trials that may give them access to therapies that may be only available to paediatric or adult populations.

Consultation item 1: rare disease threshold, seriousness of the condition

We support in principle an increased threshold for rare disease, given the number of adolescents and young adults (AYA) affected by cancers, the spectrum of cancers affecting this age group and the biological difference in cancers affecting this age group.

Each year in Australia, approximately 900 adolescents and young people aged 15-24 years are diagnosed with cancer[i], an estimated 1,000 for the 15-25 year age group supported by the Youth Cancer Services. In the region of 100 AYAs die from cancer every year (2008-2012 annual mean).

Cancer is the most common cause of non-accidental death among young people, second after injuries which predominantly caused fatal burden in this age group[ii]. Whilst a relatively small increase in reported AYA cancer incidence has occurred over the last three decades, a marked decrease in the incidence of melanoma cancers in the AYA age group masks increasing incidence in specific cancers namely colorectal, thyroid, Non-Hodgkin Lymphoma, testicular, Hodgkin Lymphoma and Acute Myeloid Leukaemia[iii] .

Cancer in AYAs is rare with 32 incidence per 100,000 for the 15-24 year age group (Australian Institute of Health and Welfare – Australian Cancer Incidence and Mortality books 2015 – decade annual mean). Additionally, the type of cancers that occur in AYAs are a mix of many types that develop in both children and adults. The three most common cancers in AYA account for 47% of all incidence (decade annual mean 2000-2009) with the remaining 34 cancers reported each accounting for between <1% and 8% of total AYA incidence demonstrating the rare nature of these cancers in this age group. The most common types of cancer in AYAs clearly differ from the common cancers either in children or in adults.

The threshold should take into consideration population sub-groups, like adolescents and young adults (see Consultation Item 4).

Consultation item 2: existing treatment and significant benefit over existing treatment
We support in principle the introduction of new criteria that aims to bring orphan products to market that treat conditions for which there is no existing treatment, or that can provide significant benefit over existing treatments.

Cancer survival in the 15-29 year age group in Australia is high, with 1-year relative survival of 95%, 88% 5- year relative survival[iv]. Those cancers most common in the AYA age group (namely thyroid, testicular, Hodgkin Lymphoma and melanoma) have the highest survival rates thereby contributing to the overall high cancer survival in this age group. However, this conceals the lower 5-year relative survival for specific cancers including brain, soft tissue and Acute Myeloid Leukaemia.

Compared with children (0-14 years) and older adults (30-39 years), relative survival for AYAs is higher (95.1% compared with 91% and 94.6% respectively at 1-year) but these data are influenced by cancers with good prognosis being more frequent in AYAs than in children again masking lower survival rates for specific, often rare cancers with many subtypes. Internationally, significantly worse survival in AYAs and young adults than in children is observed for Acute Lymphoid Leukaemias, Acute Myeloid Leukaemias, Hodgkin's Lymphomas, Non-Hodgkin Lymphomas; astrocytomas; Ewing's sarcomas; and rhabdomyosarcomas)[v]. Reasons for poorer survival in AYAs are believed to include the distinct biology of AYAs (and their response to existing child or adult treatments)[vi], rare cancer diagnosis[vii] and poorer access to clinical trials[viii].

Consultation item 3: orphan condition, medical plausibility and biomarkers
No comment.

Consultation item 4: paediatric populations
We strongly believe that consideration of adolescent and young adult populations, aged 15 to 25 years of age, as a unique population or sub-population is needed in the orphan drug program.

The incidence of cancer in AYAs is 0.8% of all reported cancer incidence and compares with 0.5% for children (0-14 years) and 2.1% for the older age group (25-34 years). It has become clear that cancer in adolescents and young adults is biologically different than in younger and older people, in addition to the unique wide spectrum of cancers affecting this age group[ix x xi]. Cancers incorporates the late onset of paediatric tumours and early onset of adult tumours; including lymphomas, germ cell tumours, brain tumours, leukaemias, melanoma, bone tumour, soft tissue sarcoma, carcinomas and other rarer tumours[xii]. With many cancers affecting AYAs, consisting of various sub types (for example, in bone cancer), this makes research and treatment more difficult for this group of young people with cancers so rare and diverse.

In addition, access to clinical trials for this age group is limited. Only 7% of 16-19 year olds and 4% of 20-24 year olds enrol in a clinical trial in Australia compared with 45% of younger children[xiii]. Reasons include the lack of clinical trials available for many cancers affecting AYAs, and age restrictions preventing AYA access to paediatric or adult focused trials.

Young people fall between the gaps in terms of having much poorer access to drugs. This could be either through the disease being rare (and therefore few trials being done in them), falling under or over the age-eligibility criteria for either paediatric or adult clinical trials, or the drugs having a high cost.

Consultation item 5: modifications to the designation process
No comments.

Consultation item 6: other considerations
No comments.

If you would like to discuss anything further, please contact me.

Kind Regards,

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Brief

About CanTeen

CanTeen is a national charity supporting young people aged 12-24 when cancer turns their world upside down. Whether it's their own diagnosis or a close family member's, CanTeen helps young people cope with the impact that cancer has on their life and connects them with others their age who are in the same boat.

We help young people dealing with:

- Having cancer themselves
- A parent, brother or sister's diagnosis
- The death of a parent, brother or sister.

CanTeen was set up by a group of young cancer patients in 1985 and we still have young people affected by cancer guiding the organisation at every level. Combined with our internationally renowned research into the emotional and social impacts of cancer, it ensures we truly understand how cancer is different in a young person's world.

Every day, another three young Australians aged 15-25 are diagnosed with cancer (around 1,000 a year) and about 75% of them will need in hospital treatment.

[i] Australian Cancer Incidence and Mortality Books [Internet]. 2015 [cited August 2015]. Available from: http://www.aihw.gov.au/acim-books/. [2007-2011 annual mean]

[ii] Australian Institute of Health and Welfare. Australian Burden of Disease Study: Impact and causes of illness and death in Australia 2011. Canberra; 2016. Report No.: Australian Burden of Disease Study series no. 3. BOD 4.

[iii] CanTeen. Cancer Data: Adolescents and Young Adults in Australia (15-24 years) - Draft Final Report (unpublished). CanTeen; 2016 October 2016.

[iv] Australian Institute of Health and Welfare. Cancer in Adolescents and Young Adults in Australia. Canberra; 2011. Report No.: Cancer series no. 62. Cat. No. CAN 59.

[v] Trama A, Botta L, Foschi R, Ferrari A, Stiller C, Desandes E, et al. Survival of European adolescents and young adults diagnosed with cancer in 2000-07: population-based data from EUROCARE-5. Lancet Oncol. 2016;17(7):896-906.

[vi] Bleyer A. Adolescent and young adult (AYA) cancers: distinct biology, different therapy? Cancer Forum. 2009;33(1).

[vii] Rare Cancers Australia. Just a little more time: Rare cancers update report. Bowral, Australia: Rare Cancers Australia; 16/03/2016. 2016.

[viii] Thomas DM, Seymour JF, O'Brien T, Sawyer SM, Ashley DM. Adolescent and young adult cancer: a revolution in evolution? Intern Med J. 2006;36(5):302-7.

[ix] L. & Zabokrtsky, K. B. Nat. Rev. Clin. Oncol. 12, 465–480 (2015); published online 26 May 2015; doi:10.1038/nrclinonc.2015.92

[x] http://cancerforum.org.au/forum/2009/march/adolescent-and-young-adult-aya-cancers-distinct-biology-different-therapy/
[xi] https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4077446/
[xii] www.cancerresearchuk.org/health-professional/cancer-statistics/teenagers-and-young-adults-cancers/incidence, accessed October 2015
[xiii] Mitchell AE, Scarcella DL, Rigutto GL, Vicky J, Thursfield VJ, Giles GG, Sexton M, Ashley DM. Cancer in adolescents and young adults: treatment and outcomes in Victoria, MJA, 2004, 180(19): 59-62.