**Cancer Voices Australia**
PO Box 5016 Greenwich NSW 2065
phone: 02 9436 1755
web: www.cancervoicesaustralia.org
email: info@cancervoicesaustralia.org

cancer voices australia

Dr Lisa Studdert
Therapeutic Goods Administration
Department of Health
PO Box 100
Woden ACT 2606
liam.sawbridge@tga.gov.au

Dear Dr Studdert

Thank you for the opportunity to comment on the discussion paper on orphan drugs.

Cancer Voices Australia is the independent, 100% volunteer voice of people affected by cancer, working to improve the cancer experience for Australians, their families and friends. We are active in the areas around diagnosis, information, treatment, research, support, care, survivorship and policy. To achieve this we work with decision-makers, ensuring the patient perspective is heard.

Cancer Voices has led the cancer consumer movement in Australia since 2000. Its networks work together on national issues identified as important, with consumers working to help others affected by cancer.

At the outset, I should emphasise Cancer Voices Australia's primary view in relation to orphan drugs is no different to our view in relation to other medicines – we want Australian cancer patients to have access to better drugs, sooner. Any proposed reforms will be judged by us against that goal.

In relation to the specific issues, we make the following comments.

**Orphan drug definition, i.e. who should be targeted with an orphan drugs program? And how can this be better reflected in the orphan drugs definition?**

As the paper outlines cancer drugs are becoming increasingly specialised, particularly as genomics advances allow for greater differentiation of cancer subtypes. The current orphan drug system is not designed for this more modern approach and it is opportune to reflect on the system as it operates now and ask whether that is the system Australia wants going forward.

Cancer Voices' view of what drugs should be defined as 'orphan' will naturally depend on the consequences that flow from that definition. If the rest of the system stayed the same, we would support allowing specialised drugs treating a subset of a cancer type to continue to be dealt with as orphan drugs and having all the advantages that implies. While cnacer drugs continue to be tailored to fewer and fewer patients, it is vital those drugs be given every advantage to come to market so that Australian patients can benefit from them.

If, however, the consequences of an orphan drug classification were to change, our support for any change in definition would depend on whether it would serve to provide Australian cancer patients

to better drugs, sooner. We would be pleased to discuss any changes you are considering in that regard.

**Is the current threshold appropriate for patient coverage?**

It seems logical that, at the very least, the threshold should increase as the population increases. We would support such a change. We would also support a model, such as that presented as an option, which uses a percentage of the population instead of a static number. This appears to be used successfully in other jurisdictions. We note that the ratios applied in other jurisdictions are significantly more generous than those used by the TGA (Discussion Paper Table 2).

**Are changes needed to the charging model?**

As the paper outlines the current charging model is such that orphan drug submissions are "paid for" by non-orphan drug submissions. While the paper outlines some interesting information about how many orphan drug applications a company has made, it does not explain, for example, how much other money those companies pay in relation to other drugs, or how many times a company has sought to apply orphan drug status to the same drug for multiple indications.
Unfortunately, without knowing more about the way the system actually works and companies operate now, we are unable to make a comment on what charges should be made to the funding model. Again, we would be pleased to discuss any proposals in relation to this with you.

We note also there are other issues which might be considered by the TGA, which might flow from an orphan drug designation such as market exclusivity, which do not appear to be covered by this discussion paper but might perhaps be included in a review of the Australian orphan drug system.

We thank you again for the opportunity to put forward the voice of Australian cancer patients and their families.

Yours sincerely

Bridget Whelan