Submission to the Senate Inquiry into the Availability of new, innovative and specialist cancer drugs in Australia.

Introduction

My name is Peter Carr & I was diagnosed with Waldenstrom's Macroglobulinemia (WM) in 2004, which is a non-Hodgkin's Lymphoma. It is a very rare cancer & thus has a very small number of patients in Australia. Although I don't have the exact numbers in Australia, it is probably less than 1,200 patients with this cancer. There are approximately 15-20 new cases each year.

I belong to the International Waldenstrom's Macroglobulinemia Foundation (IWMF) a US based charity that provides support to WM patients & their caregivers. In addition it funds research into WM.

I also belong & help run the Australian patient support group, affiliated to the IWMF, which we have called WMozzies. We are assisted by the Leukaemia Foundation to provide patient support & meetings for WM patients & their caregivers.

The Problems

- Drug companies are not keen to list drugs for WM with the PBAC, because of the cost & paperwork required for even applying for the listing.
- The Australian requirement for supporting documentation seems excessive as it is duplicating work done in overseas countries.
- The entire treatment of WM is moving towards targeted therapies. These targeted therapies may differ from case to case and there could be many of them. The PBAC system is not set up to deal with rapidly developing & changing therapies.
- Lack of special rules for rare cancers. The FDA in the US has given WM an orphan cancer designation & developed much more streamline processes to get drugs approved for WM.

The Solution

- Setting up of a Cancer Drug Fund, similar to the one in the UK, as a temporary measure, until the PBAC structure is modernised. This fund in the UK funds drugs that are not covered by the UK equivalent of the PBAC.
- Complete overhaul of the PBAC approval process to streamline the process and reduce the disincentive of cost for Drug Companies to apply for drugs that have only a limited market in Australia.
- Endeavour to harmonise the supporting documentation so that documentation prepared in other countries can be used in Australia.
- Specifically adopt the same process as the FDA for handling rare cancers such as WM, which has recently seen the FDA approve a new drug called Imbruvica specifically for WM, the first drug specifically approved by the FDA for WM. This drug is the first of the targeted therapies & there is many more in development. The FDA did this by giving Imbruvica a "Breakthrough Therapy Designation".

Conclusion

I have deliberately kept this very brief as I have no doubt that you have much material to read. However I urge you to overhaul the PBAC system in its entirety BUT specifically in the case of how the PBAC handles rare cancers, such as WM.

I also urge you to be mindful of the incredible number of new treatments that have been developed & are being developed. The PBAC system needs a complete overhaul to handle what is indeed a brave new world for cancer treatments.

Kind Regards

Peter Robert Carr