Personal submission by Andrew Warden to
Senate Community Affairs References Committee for inquiry and report on availability of new, innovative and specialist cancer drugs in Australia.

My submission covers:

- Personal, cancer diagnosis and treatments
- Timing and affordability of access for patients
- Operation of the Pharmaceutical Benefits Advisory Committee (PBAC) and the Pharmaceutical Benefits Scheme (PBS) in relation to such drugs, including the impact of delays in the approvals process for Australian patients
- Impact on the quality of care available to cancer patients
- Related matters.

Personal, cancer diagnosis and treatments

Personal

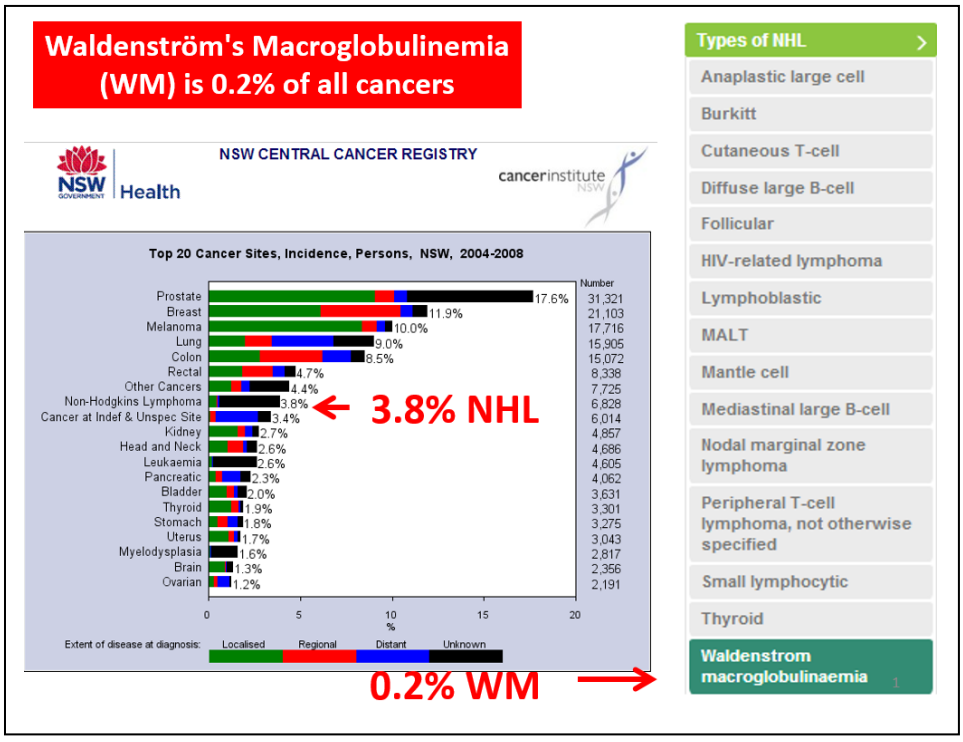
I am a retired Chartered Accountant and a Deputy Captain in the NSW Rural Fire Service. I live at [redacted]. (Boat access only community on western foreshore of [redacted]). Married 2 children 6 grand children (ages 10 to 3)

Andrew Warden at Parliament House with Co-chairs Parliamentarians supporting Cancer Causes 2014

Andrew Warden bush firefighting in Blue Mountains in October 2013

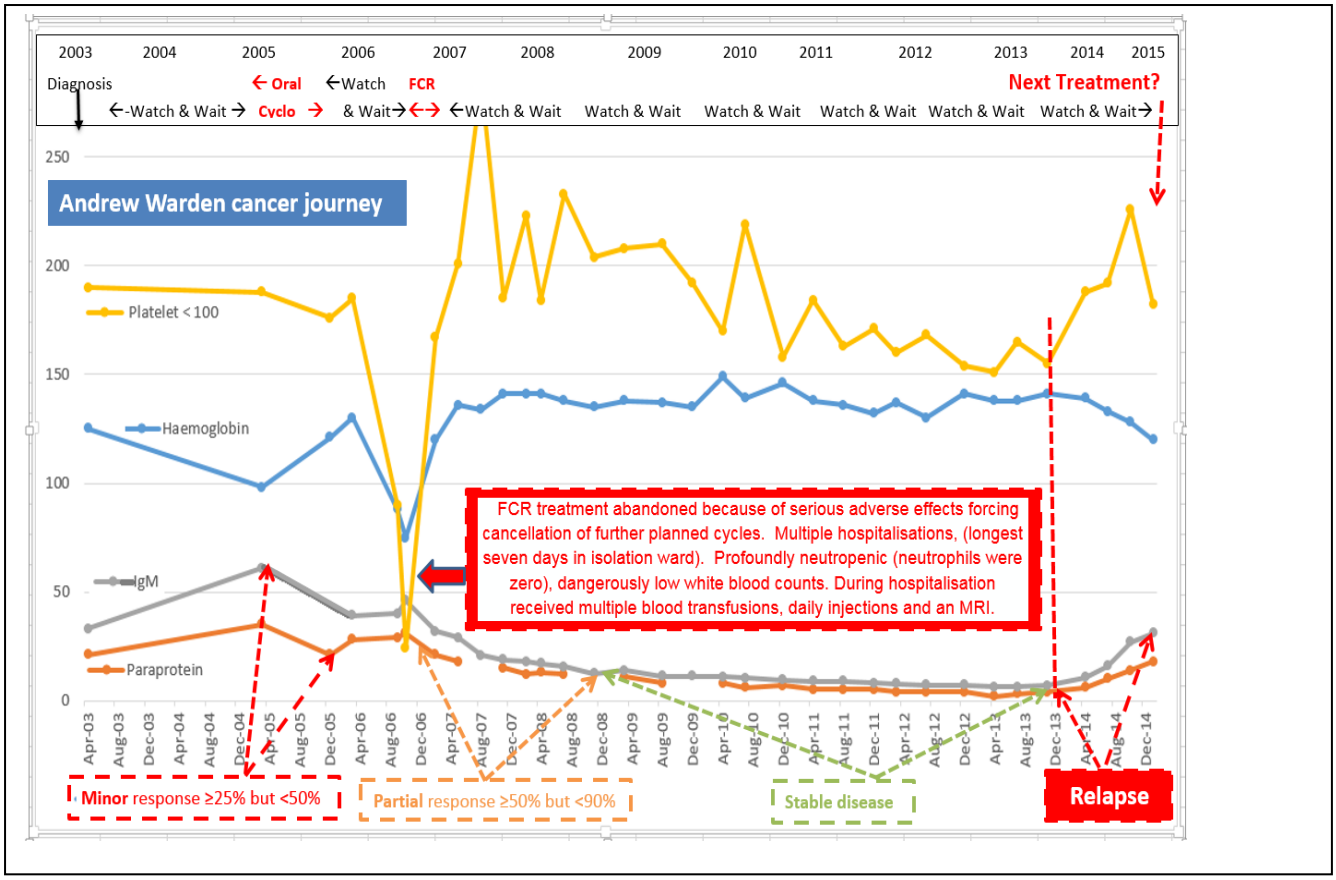
Cancer Diagnosis

In April 2003 I was diagnosed with Waldenström's Macroglobulinemia (WM) which is a type of Non-Hodgkins Lymphoma (NHL). WM is rare being 0.2% of all cancers and NHL is 3.8% of all cancers. WM is incurable but treatable.



Treatment experiences

I have had treatments in 2005 and 2006-07. In 2005 I had Cyclophosphamide. In 2006-07 my treatment was with Fludarabine, Cyclophosphamide and Rituxan (FCR). The FCR treatment had to be abandoned because of serious adverse events forcing cancellation of further planned cycles. I had multiple hospitalisations, the longest being seven days in the isolation ward. I was profoundly neutropenic (neutrophils were zero) and had dangerously low white blood counts. During hospitalisation I received multiple blood transfusions, daily injections and an MRI. It was not established whether the adverse events were caused by Fludarabine or Rituxan.



Timing and affordability of access for patients

As a WM patient in relapse and requiring further treatment I am not getting timely and appropriate access to new medicines that I need.

My Haematologist is now deciding on my next treatment. Recent medical experience has shown that retreating with FCR there is a 20% chance of transformation in my blood cancer. Although FCR has PBS funding it is no longer considered suitable because of the serious adverse events I previously experienced with FCR. The adverse events may have been caused by a possible intolerance to Rituxan which is a chimeric (mouse/human) monoclonal antibody. The overseas only available alternative in these cases is Ofatumumab which is fully humanised. Under the Australian health regime Ofatumumab is not available here for WM.

Access to the overseas superior treatment options which take into account recent medical advances is important. It fair and reasonable that my access should be in line with the treatment recommendations of world experts for WM patients (IWWM-7 consensus workshop panel 2012 Newport Rhode Island US of 25 WM experts from leading medical and research organisation across US, UK, Germany, Greece and Italy - Blood, 28 August 2014 * Volume 124, Number 9).

The consensus of leading world experts identifies WM treatments including IMBRUVICA, Idelalisib, Ofatumumab, Velcade and RIBOMUSTIN. I do not have access to these treatments. There are Australian clinical trials (with limited patient intakes) for all these treatments except Ofatumumab which is only for Chronic Lymphocytic Leukaemia (CLL). My Haematologist late last year unsuccessfully sought my participation in the IMBRUVICA clinical trial. I did not then meet the specified criteria as my relapse had not then reached the stipulated level. The trial is now closing before my condition is within the defined criteria, so my chance has passed.

Only RIBOMUSTIN has TGA approval for WM. It appears that the system places me with a rare cancer at a disadvantage to those with more common cancers. There is TGA approval for the use of Velcade, Idelalisib and Ofatumumab for CLL / SLL but not WM. It is unfair that the present system gives authorisation and access to some cancers but not mine.

None of the overseas identified best treatments have PBS funding approval. The March PBAC meeting is considering PBS funding approval for RIBOMUSTIN but experience shows that there is only a 1 in 5 chance of PBAC approval. Clinical trials in the US and Europe and US Food & Drug Administration (FDA) investigations have established that RIBOMUSTIN has significantly better outcomes in terms of progression free survival and less adverse events. The UK Clinical Drug Fund provides funding for RIBOMUSTIN.

Operation of the PBAC and the PBS in relation to such drugs, including the impact of delays in the approvals process for Australian patients

Our regulatory system although robust is clouding out consideration of my requirement as a patient with a rare cancer to the treatments which are available overseas. The current system is cumbersome, lengthy and constrained by economic imperatives that are not representative of the international picture. The organisations involved, including PBS and PBAC, were formulated almost 60 years ago and don't provide for due consideration of my treatment needs. The regulatory system is not equipped to respond quickly to the advances being made in cancer treatment. I need a system that recognises the significant advances in cancer treatment and provides access for me and all Australians to these innovative medicines for the patients who need access to life preserving treatments.

There is need for a better, quicker and more affordable way for me and other cancer patients to get the drugs needed. We can't wait the years that our present approval process takes, nor can we afford to pay the full unsubsidised costs. We understand that new cancer drugs are costly but our approval processes are slow and not designed to cater for the changes in cancer research technologies.

The Australian PBAC system seems too rigid, slow to react and out-dated in comparison to other comparable processes overseas (e.g. the UK). The system needs to keep pace with rapid medical advancements and efficiencies adopted in other countries.

Points needing consideration to improve efficiency include

- Having more effective and timely consumer input within the PBAC and MSAC processes
- Having at least one other consumer representative on the PBAC, preferably with a cancer background
- Establishing a consumer sub-committee the PBAC can call on for information regarding specific patient conditions
- Having more frequent revisions to PBAC system and processes to accommodate medical advancements,
- Holding meetings more frequently to fast-track decisions
- Having a faster process to get Australians onto the cancer drugs which will help them, possibly using a “special track” process.
- Being able to assess cancer drugs’ effectiveness and impact on quality of life in real-life use (not just in clinical trials) using post marketing surveillance
- Having an agency or registry to collect and link data about real-life benefits and adverse events
- Examining cancer specific drug access solutions reached in other countries to see which elements could work in Australia -e.g. UK Cancer Drug Fund
- Using accredited overseas approvals in appropriate cases to enable faster access to new cancer drugs

Impact on the quality of care available to cancer patients

My life would be improved by PBS funding availability of proven overseas treatments for my rare blood cancer. It is reasonable for me and other Australians to expect access to cancer treatments which have been established to be the best by the consensus of world specialists. The improved access would free my family from much worry and concern about my life span and ability to have a full role with my children and grandchildren. I wish to have access to the needed drugs to restore my strength and enjoy an active life. I want to resume an active role as a volunteer fire fighter in the NSW Rural Fire Service. My relapse has prevented my continuing in strike force activities since the Blue Mountains fires.

The financial benefit is major of having novel treatments listed for PBS funding such as RIBOMUSTIN now being considered by the PBAC. The cost burden without PBS funding for the best treatments would create financial difficulties for me and many others putting the desired best treatments out of reach.

It is important for me and all Australian patients to have access to new medicines, including cancer medicines and innovative treatments, and for these new medicines to be listed on the PBS in a timely manner. Cancer medicines and innovative new treatments over the past thirty years, have made a significant contribution to the quality of life and life expectancy of cancer patients. While the incidence has grown rapidly, survival from cancer has improved significantly, with five-year survival from all cancers combined rising from 46% in 1982-1986 to 67% in 2007-2011. The Australian Institute of Health and Welfare (AIHW) report, *Cancer in Australia in brief 2014*, found that the overall mortality rate from cancer is expected to have dropped by 20 per cent in the last three decades - from 209 deaths per 100,000 people in 1982, to 168 deaths per 100,000 people in 2014.

Related matters

The UK has addressed the cancer drug access and timing issues. They operate a Cancer Drug Fund (CDF) as an interim solution pending a total system overhaul. It seems to me that such an initiative should be introduced in Australia. The UK CDF was established in 2011 following patient and clinician complaints regarding delays in access to new cancer medicines. It continues to enjoy strong stakeholder support with both sides of UK politics committing to maintain and even increase its funding. Notwithstanding this, CDF has faced bureaucratic criticism regarding its lack of adherence to cost-effectiveness principles. This is claimed to undermine established policies and processes used by the UK National Institute for Health and Care Excellence. This argument does not stand up from the viewpoint of patients who would otherwise be missing out on needed treatments because of slow cumbersome outdated assessment procedures. Surely patients in need should not suffer because the monitoring system has not been updated by the responsible health authorities.

For those with rare and less common cancers, a CDF type interim system could provide a way to quickly access treatments that are already listed on the PBS for other cancers (at the request of their physician).

Australian patients it seems are missing out on new cancer medicines and relying on older alternatives compared to many other countries. A recent UK study has found that Australia ranked 12th out of 13 countries on the usage of cancer medicines approved within the past 5 years, only in front of New Zealand, and down from 10th in 2009.

It is of critical importance that the outcome from the Senate Enquiry ensures that there is fulfilment of the recent commitment given by the Department of Health to a Senator's question. "The Australian Government is committed to ensuring Australians get quicker access to medicines, no matter what their condition."