Cure Brain Cancer
FOUNDATION
Many minds, one purpose

CURE BRAIN CANCER FOUNDATION: GOVERNMENT SUBMISSION

Submission to the Senate Committee on Community Affairs
Inquiry into the availability of new, innovative and specialist cancer drugs in Australia

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Introduction

At present people diagnosed with brain cancer will usually die within 16 months. A diagnosis of brain cancer is terminal. If a patient is lucky enough to survive they will often have severe, multiple deficits with very high care needs. In Australia, more children die from brain cancer than any other disease and more people under 40 than any other cancer. Brain cancer is a devastating disease, for patients, families and carers. Individual patient care costs more (to Government) than any other cancer.

Currently there are few drugs available to treat brain cancer. The main PBS-listed chemotherapy, Temodal (temozolomide), has now been shown to only to work in approximately 30% of patients with a glioblastoma (a deadly grade IV brain cancer), who are MGMT positive. The overall benefit to patients is minimal: on average 2 months extra life. Thus, the major therapeutic options to treat brain cancer are currently surgery and radiotherapy. These modalities, while prolonging life, are not a cure and often result in severe deficits and iatrogenic disease.

Cure Brain Cancer Foundation (CBCF) is the peak brain cancer research and patient advocacy organisation. We are tackling the paucity of brain cancer medicines in two ways:

- A significant, increasingly global, research collaboration, to accelerate new treatment options for patients.

- A concerted move towards a 'personalised medicine' approach, whereby tumour genetics are established early on and high-throughput screening of existing medicines (many of which are currently PBS listed for other indications) occurs. If any of the screened drugs show activity against an individuals' tumour, then this information is conveyed to the treating oncologist (and hopefully patient) to be used for (most likely off-label) treatment.

Patients need improved access to off-label and experimental treatment options that show early efficacy instead of complying with existing treatment protocols that we know are ineffective in up to 70% of cases. Patients also need access to new treatments immediately when they become available through FDA approval, not up to five years later, when they might possibly be PBS-listed (reimbursed) in Australia.

Children and adults living with brain cancer must be shown compassion in decision-making around access to treatments. It is our collective duty to protect and defend the rights of the most vulnerable Australian citizens from any politicised factors that may hinder appropriate early access to effective brain cancer medicines.

It will be unconscionable if, after all the hard work of researchers, patient advocates, adults and children with brain cancer, families and carers, all enduring the devastating impact of brain cancer, that the Government or the pharma industry in any way delays availability of new treatments to patients. There is no reasonable argument for such a worst-case scenario to play out.

Desired effect

Significantly earlier (and affordable) access for people living with brain cancer to any drug (new, innovative, or off-label, expensive or cheap) agreed as part of the patients' treatment plan.

Key recommendations

1. **Make it simpler, easier and quicker for patients (and less stressful/fearful/legally concerning) for doctors to access off-label medicines**
   a. People diagnosed with brain cancer and many other cancers are increasingly using the companies such as Caris and Foundation Medicine, and services (such as Caris's Evidence-guided tumour profiling service) to ascertain tumour-specific treatment recommendations based on the genetic profile of their tumour. Often the recommended treatment approach will be for off-label use of an already PBS listed drug/s. There may also be a potential recommendation of a drug that is not available in Australia that must be accessed on compassionate grounds
   b. Suggested approach:
      i. Quantify patient/carer/parent propensity for risk and ensure access to off-label medicines in timely manner.
      ii. Ensure full and frank disclosure of potential impact (both positive and negative).
      iii. Obtain signed consent to off-label treatment plan.
      iv. Indemnify prescribing doctors' potential liability issues for terminally ill patients with appropriate informed consent.
      v. Set up tracking and monitoring (data capture to assess impact and inform future decisions), possibly through a registry.
      vi. Subsidise the cost of recommended medicines.
   c. Provide early access to experimental treatments through a scheme (with a lower burden of proof) similar to the "Promising Innovative Medicine" status as part of the UKs 'Early Access to Medicine' scheme and the US FDAs "breakthrough status".

2. **Ensure significantly earlier access for people living with brain cancer to new drugs**
   a. Use FDA approval as the basis for Australian approval (reducing time to TGA marketing approval to a maximum of 3 months post FDA).
   b. Introduce Conditional PBAC approval and PBS listing (with mechanisms still to be decided), immediately upon TGA approval, whereby PBAC cost-efficacy evaluation and PBPS negotiations are done in parallel rather than in sequential order (we envisage some sort of balance between compassionate use / access, with defined usage parameters for pharma to be able to guarantee supply) with speedy arbitration should PBS listing be denied.
   c. PBAC approval mechanisms must be mandated to take into account and appropriately weight the potential for a new medicine to reduce cost throughout the healthcare system (taking into account both Federal and State healthcare system cost savings).

3. **Ensure equality of access to off-label use of currently approved medicines and new medicines for children with low prospects of survival and/or high morbidity, including brain cancer**
   a. If 2(b) is adopted, ensure pharma companies are mandated to provide compassionate use of these medicines regardless of whether they have evidence of use in children, with concomitant collection of data through a registry process.
   b. Provide a legal indemnity solution if this is cited as a barrier by industry.

4. **For brain cancer and other low 5-year survival cancers, adopt the approach pioneered with HIV medicines whereby positive Phase II results enable the drug to be made available for compassionate use, with data collection through a registry.**
   This approach is currently being used in Germany and the UK for ICT107, an experimental vaccine treatment for brain cancer. It is not available in Australia.

Discussion

This submission succinctly addresses key recommendations with the following discussion points. More context will be presented to the Senate Committee to support this submission upon request.

While we are hopeful that this Senate Enquiry will result in action being taken to reduce the time taken to make new medicines available on the PBS, we remind the Government of its moral duty to serve the needs of its constituents, (in this case patients), and close the gap between industry and Government through strategic and strong leadership decisions.

We understand the complexities the Government is dealing with given that industry is driven by what can be termed its 'Global Pharmaceutical Pricing Imperative'. This methodology, used by pharma industry HQs, to ensure maximum profit is made from the drugs they produce, dictates first marketing new drugs in the largest ($ value) markets, where they can achieve highest prices. Thus, drugs approvals are often sought in the following order: the US, Japan and EMEA, before working through the rest of the world on a similar, if not so country specific, basis.

Given that Australia is a small market that demands significant price reduction more often than not, through the PBAC process for PBS listing, it features very low down on the big pharma 'to do' list. In essence, this system ensures consumers in large markets with high prices (like the US), subsidise consumers in small markets demanding lower prices, like Australia. The trade off is (most unfortunately for patients with life-threatening diseases) delayed and often extremely delayed access. Delayed access for people living with brain cancer often means certain death.

We also believe that Government/PBAC is driven by keeping the cost of the PBS as low as possible. This is essentially achieved through what many in industry believe is a flawed system to assess cost-effectiveness of new drugs. While drugs not listed on the PBS are available for prescription to patients, following TGA approval, in reality they are often far too expensive, without reimbursement, for most patients to afford.

In effect, this is a Catch 22. Government demand for the lowest price ensures delayed access through pharma industry mechanisms to ensure profit maximisation, while industry demand for the highest price ensures that Government delays access to defray and delay cost to the PBS through cost-effectiveness hurdles.

Patients however, are increasingly demanding better access especially when their choices are few and the risk of dying is high (as is too often the case for patients with brain cancer). A solution needs to be found whereby patients can access new drugs in an affordable manner while industry negotiates with PBAC. To this end we recommend that **the primary PBAC objective should be to ensure access to patients within agreed and specified timelines** rather than to keep the cost of the PBS down. Greater transparency on both sides will help achieve this.

Many believe that the current system is not working and is failing those it is supposed to help – patients. Therefore to achieve a positive outcome for patients, the current system needs to be disrupted (as it is about to be, through the impending personalised medicine revolution), and innovative ways to both change and improve it must be considered and implemented.

From a patient advocacy perspective it feels that neither Government nor industry really appreciate what is at stake. Australians – our children, mothers, fathers, sons and daughters, brothers and sisters are dying every day, while access to potentially lifesaving medication is withheld due to bureaucracy.

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Founded by Associate Professor, Charlie Teo in 2001, and headquartered in Surry Hills, NSW, Cure Brain Cancer Foundation is the peak brain cancer organisation in Australia, with 24 employees in NSW, Victoria and Queensland. Partnering with the research and brain cancer communities, we are developing a national agenda (and influencing the global agenda) for brain cancer research, having funded more than $14 million worth a research projects since inception.

CBCF is focused on achieving its mission (to increase brain cancer 5-year survival from the current 20% to 50% by 2023) though a disruptive research strategy that focuses on adaptive trials, personalised medicine and facilitation of collaborative research and patient advocacy. Brain cancer affects children as well as adults, so CBCFs mandate crosses the paediatric/adult health system systemic divide.

We recognise that to solve a complex problem like brain cancer we need to do things differently; to think laterally and find smarter solutions that allow us to make breakthroughs much faster than traditional research methods allow. We aim to disrupt, challenge accepted practice and innovate.

In 2014 CBCF donated more than $5 million to brain cancer research including $2.2 million for nine research projects across three Australian states, four countries and twenty-four institutions, as well as $2.8 million to further fund the Brain Cancer Discovery Research Collaborative (BCDC) that operates across four states.

For more information, please contact:

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