

CURE BRAIN CANCER FOUNDATION: GOVERNMENT SUBMISSION

**Cure Brain Cancer Foundation submission to the Senate Community
Affairs Legislation Committee inquiry into the Medical Research
Future Fund Bill 2015**

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Executive summary

Cure Brain Cancer Foundation, as the peak organization for brain cancer research, advocacy and awareness in Australia, broadly supports the introduction of the MRFF and the associated Bill.

We believe that the MRFF offers a visionary opportunity to increase the effectiveness and global standing of medical research in Australia through a bold and disruptive innovative approach, with the aim of dramatically enhancing health outcomes in the shortest time. This should include delivery of personalised medicine and a pan-cancer approach to funding cancer research.

This is unlikely to be achieved using 'old' structures and thinking. It will need new people, with new thinking, unfazed by the prospect of overcoming what is likely to be significant resistance from established hierarchies.

We also believe that mechanisms set up for the disbursement of funds should prioritise areas of **unmet medical need** for the Australian community. 'Unmet medical need' can be defined as those diseases where 5-year survival is low, mortality and morbidity is high, treatments and treatment options are few, with inadequate efficacy. Brain cancer, which kills more children than any other disease and more adults under 40 than any other cancer, fits into this category.

The US recently passed the 21st Century Cures Act and we encourage that this be reviewed to ascertain areas of synergy and ideas that can be adopted to enhance the scope, operational and innovative remit of the MRFF.

MRFF needs to be a bold, disruptive medical research initiative with funding preferentially allocated to health priorities based on unmet medical needs such as brain cancer

The increase in research funding through the MRFF is positive but should not lower the bar

The MRFF innovation mandate needs to be bold and unwavering in its commitment to drive the use of current/future technologies to dramatically enhance health outcomes in the shortest time

Funding priorities are key and should be based on unmet medical needs and set through community consultation

Brain cancer is an unmet medical need killing more children than any other disease and more people under 40 than any other cancer

The increase in research funding through the MRFF is positive but should not 'lower the bar'

The MRFF is a positive initiative for Australian medical research

The MRFF presents an opportunity to empower researchers and enable new technologies and approaches to be embedded in the health system

The MRFF should not 'lower the bar' but fund more innovative projects

The MRFF is a broadly positive development offering Australian medical research the opportunity to further enhance its international standing and competitiveness. With the potential over time for substantial increases in medical research funding, it is imperative that the opportunity is taken to develop new approaches to funding researchers, laboratories and research institutions - one that encourages collaboration and includes significantly enhanced sharing of data to genuinely improve health outcomes in the shortest time.

Enabling researchers to spend more time in the lab and less time writing and pitching for funding is critical as is a forward thinking approach with a view to embedding new medical practice into the health system extremely quickly.

Just because more funding is available does not mean that the bar should be lowered. Raising expertise and capacity, especially in areas of obvious need, should be a priority. It is likely that there will be a significant gap between what is needed to address priority areas of unmet medical need and current allocation of resources.

The MRFF innovation mandate needs to be bold and unwavering in its commitment to drive the use of current/future technologies to dramatically enhance health outcomes in the shortest time

Australia has an opportunity, through the MRFF, to enhance its global standing in research and innovation and attract more investment

The definition of innovation should be enhanced and a bold disruptive innovative approach built into the MRFF DNA - this should heavily influence Advisory Committee member choices and weighting

The MRFF strategic plan should be revised every 18 months to keep up-to-date with new and emerging technologies and thinking

It can be argued that the definition of 'innovation' provided in the Bill is unnecessarily narrow: *'medical innovation means the application and commercialisation of medical research, and the translation of medical research into new or improved medical treatments, for the purpose of improving the health and wellbeing of individual'*

The very recent, bipartisan, 21st Century Cures Act in the US has sought to address this through specifically embracing new tools like adaptive clinical trial design, biomarkers and new models for research commercialisation.

With the rate of disruptive innovation in health and medicine happening faster and faster, it would be appropriate to adopt a similar strategic review pathway as the US and review the strategic plan every 18 months. This will help ensure that Australia does not fall behind in the global medical and health innovation stakes.

We are concerned that, given the focus on innovation, that there is not enough emphasis in the Bill on new approaches, both in design and function. Given that brain cancer survival has hardly moved in the last 30 years, Cure Brain Cancer Foundation's ten-year mission, to increase brain cancer survival to 50% by 2023, is time critical. People living with brain cancer cannot afford the luxury of having things done the same as they have always been done, and proved not to work (this was defined by Einstein as 'insanity', rather than innovation).

Innovation, in our book, means disruption – challenging the norm. To this end we would encourage the Advisory Board to include disruptive thinkers, who are not afraid to fail and adopt (relatively) short timeframes for success. They should seek to both energise the medical research community and to be bold, encouraging / mandating open source sharing of data and re-framing criteria for researcher and research success. Time is critical. Every day lives are being lost. Innovation in this sense is likely to be a tall order for established government research structures and personnel. A new approach is needed - a riskier approach, but one that may pay enormous health and financial dividends.

The recent US 21st Century Cures Act noted that the foundation for 21st century medicine cannot be built on top of decades-old structures. Genomics, data analytics, health information, and other constantly evolving data sources, technologies, and platforms have already fundamentally transformed many aspects of patient care and health care delivery in the US and they doing so, albeit at a slower pace, in Australia.

For instance, a major advantage of adaptive trial design is shorter time frames and Master Protocols, lower costs and new incentives for blockbuster drugs to be used in rarer diseases / unmet medical needs.

An example of a structural change is a pan-cancer approach to research, focusing on tumour mutations, rather than tumour site. Although brain cancer may warrant a special case in this regard, since most treatments cannot cross the blood-brain barrier. In this case a pan-brain disease approach may be warranted, including all tumours occurring in the brain along with other brain diseases. Another example is the broad adoption and role out of personalised medicine.

A data sharing framework, unlocking data sitting in siloed healthcare facilities across the country and enabling patients who want to play a more proactive role in finding better treatments or a cure for their disease, to do so in a responsible manner that

continues to protect their privacy, are all challenges that need to be addressed. For this CBCF believes that the 'appropriate balance' with regard to the Advisory Board is key. It should be heavily weighted on the side of innovation and commercialisation and help enable a focus on bold disruptive innovation to be built into the MRFF DNA. Of major concern is that the Advisory Board may, when chosen, reflect 'old practice' rather than 'new thinking'. It is most unlikely that those steeped in old ways of operating in the health, medical and research spheres, will be able to adequately contemplate or to be comfortable with new approaches, many of which will challenge currently accepted wisdom, practice and ethics.

**Funding priorities must be set through community consultation
and be based on unmet medical needs**

Government should focus funding on areas of 'unmet medical need' and address health inequities

Patient perspectives should be taken into account

Broad and ongoing community consultation is critical to enable appropriate priority setting

Funding should be prioritised based on **unmet medical needs**. These are conditions for which no satisfactory method of prevention, diagnosis or treatment exists, with low survival and/or low quality of life. They have traditionally been passed off as 'too hard' and/or affecting 'too few'.

For example, mortality from rare / less common cancers is now as high as mortality from more common cancers. This mostly reflects the past and current prioritisation of common cancers and the funding and research focus that this has facilitated. These more common cancers such as breast and prostate in adults, and leukaemia in children, now have very high 5-year survival (>90%).

Children with unmet medical needs should be prioritised above adults with unmet medical needs. Our children are our future and they deserve to live long, fulfilling lives, realising their potential for the benefit of all Australians.

Indeed, we believe that unmet medical needs for children, such as brain cancer should be allocated a specific 'earmarked' proportion of MRFF funding on an ongoing basis. It is imperative to note, that many children who do survive brain cancer for more than 5 years, are often severely compromised, needing constant care.

It is important that the determination and setting of priorities within the MRFF takes into account past and current medical inequities as these have long lasting effects on specific research capabilities and capacity in Australia.

However, as noted recently by the US President's Council of Advisors on Science and Technology, *'the current system in many ways discourages investment in therapies*

for scientifically complex diseases with longer development times. Congress has been encouraged to tackle this problem since today's patent framework often makes it economically unviable to bring such therapies to market, even when they have shown early promise'.

CBCF has already made representations to the Australian Government showcasing the 'Catch 22' within the current NHMRC funding process for brain cancer, (a very low 5-year survival, rare cancer) whereby it has continued to attract comparably low funding (when compared with more common cancers with high 5-year survival). We strongly believe this is because quality researchers and research is to a significant extent a result of higher funding and vice versa: high funding attracts more, higher quality, researchers and research.

CBCF believes it is important that the mechanism for determining priorities involves community consultation and is transparent in outcomes, with regular review. The process needs to avoid potential respective 'natural' biases of committee members. It should include consumer representation, from suitably qualified individuals, who can represent the whole community. However, consumer representation on the committee does not replace the need for broad community consultation in the setting of priorities.

We also believe that it would be very helpful to gather patient perspectives (for example people living with brain cancer, their carers and families) to help determine priorities. While consumer representation is very valuable, hearing directly from patients brings a unique and current perspective from those directly affected.

Brain cancer is an unmet medical need killing more children than any other disease and more people under 40 than any other cancer

Brain cancer survival is very low ($\approx 20\%$) which has hardly moved in 30 years

We don't know what causes it, cannot prevent it and treatments are few (with high negative impacts)

The burden of care and societal cost is very high, with high morbidity and low QOL for those 'lucky enough' to survive

As a clear area of unmet medical need, brain cancer, which kills more children than any other disease and more people under 40 than any other cancer, deserves an immediate increase in focus and funding.

With surgery and radiotherapy the mainstays of treatment, (when ideally they would be the last resort), the few people 'lucky' enough to survive for 5 years are often severely compromised, needing high levels of care. Brain cancer is crying out for new effective treatment options that minimise harm and substantially increase survival.

Cure Brain Cancer Foundation

Founded by Professor, Charlie Teo in 2001, with its HQ in Surry Hills, NSW, Cure Brain Cancer Foundation is the peak brain cancer organisation in Australia, with 26 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 committed \$11 million worth of research funding in the last 12 months. This includes \$2.2 million for nine research projects across three states (including NSW), 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.

CBCF is focused on achieving its mission (to increase brain cancer 5-year survival from the current 20% to 50% by 2023) through facilitation of collaborative research, patient advocacy and awareness. 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.

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