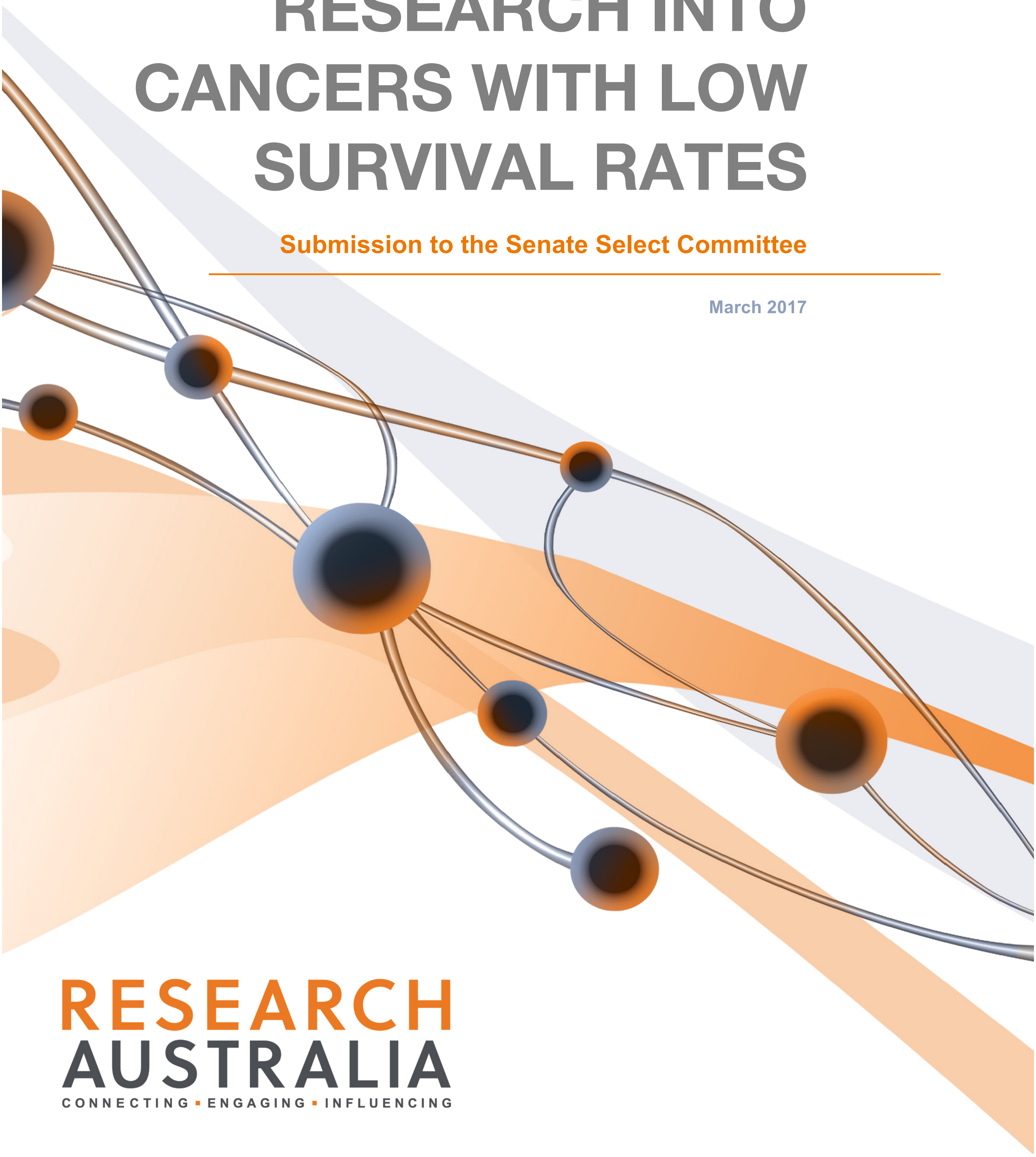
FUNDING FOR RESEARCH INTO CANCERS WITH LOW SURVIVAL RATES

Submission to the Senate Select Committee

March 2017



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ABOUT RESEARCH AUSTRALIA

Research Australia is an alliance of 160 members and supporters advocating for health and medical research in Australia. Research Australia's activities are funded by its members, donors and supporters from leading research organisations, academic institutions, philanthropy, community special interest groups, peak industry bodies, biotechnology and pharmaceutical companies, small businesses and corporate Australia. It reflects the views of its diverse membership and represents the interests of the broader community.

Research Australia's mission is to make health and medical research a higher priority for the nation. We have four goals that support this mission:

- A society that is well informed and values the benefits of health and medical research.
- Greater investment in health and medical research from all sources.
- Ensure Australia captures the benefits of health and medical research.
- Promote Australia's global position in health and medical research.

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SUBMISSION TO THE SENATE SELECT COMMITTEE

Introduction

Research Australia welcomes the opportunity to make a submission to this Inquiry. Our membership is drawn from across the whole pipeline of health and medical research, including all diseases areas and disciplines, and the full range of research organisations from universities to multinational corporations. Many of the Terms of Reference have relevance beyond brain cancers, cancers with low survival rates and low incidence cancers; our submission approaches the Inquiry from this broad perspective and considers the following.

Government funding available for health and medical research is finite. While cancers with low survival rates are undoubtedly worthy of more funding, any increase in funding for one area has implications for the funding available to other disease areas.

Research Australia acknowledges that NHMRC competitive grant funding has become even more competitive in recent years and that many research applications that are worthy of funding are not funded because the number of applications eligible to be funded well exceeds the available funding.

It must be noted that improvements in survival are related to global research rather than specifically to the volume or subject of Australian research, and it is important that Australian researchers and patients have access to, and contribute to this global effort. This is particularly relevant for low incidence cancers and other rare diseases, because the number of patients in Australia is likely to be low and appropriate research collaborators are in many cases likely to be based overseas. Thus, when allocating funding we also need to consider the level of funding available and the research activity undertaken globally.

Research Australia also suggests there is merit in looking at approaches taken by other countries in addressing similar dilemnas. This includes strategies such as rare disease policies that have been established in more than 20 countries as a means of providing a policy framework for a considered and comprehensive approach to the provision of research, diagnosis and access to treatment for rare diseases. In an area where the only available therapies are often experimental, a single policy that brings research, diagnosis and treatment together is valuable.

These observations are explored further in the body of this submission.

Summary of Recommendations

Term of Reference	Recommendation
a.	**Research Australia submits that to address the influence philanthropic and other non government funding can have on the allocation of Government funding through competitive grant programs, the Government should fully fund the indirect costs of research for which it provides competitive grant funding.**
b.	**Research Australia submits that existing initiatives to improve clinical trials should be supported and their success in improving access to, and the conduct, of clinical trials for: a) all diseases; and b) brain cancers and other low incidence cancers, should be evaluated before any specific funding models are developed for clinical trials for brain cancers and other low incidence cancers.** **Research Australia submits if the aim is to support fundraising from the public, funding for these awareness campaigns should remain the responsibility of the patient groups. It would be more efficient and effective for the Commonwealth to allocate already stretched funding directly to more health and medical research.** **Research Australia further submits using Commonwealth funds to increase public giving is through research funding programs that match Government funding with funds raised from the public.**
c.	**Research Australia encourages consideration of the development of an appropriate policy framework for rare diseases as a means of addressing the Inquiry's Terms of Reference.** **Research Australia submits that new funding would be required to support the provision of diagnosis services, specialised health care and research if such a policy was to be successful.**

Response to the Terms of Reference

Research Australia's submission addresses each Term of Reference in turn.

The impact of health research funding models on the availability of funding for research into cancers with low survival rates, with particular reference to:

a. the current National Health and Medical Research Council funding model, which favours funding for types of cancer that attract more non-government funding, and the need to ensure the funding model enables the provision of funding research into brain cancers and other low survival rate cancers;

There are many sources of funding for health and medical research in Australia, including Commonwealth, State and Territory Governments, philanthropy and the private sector.

ABS data reveals that in 2014, the higher education sector alone expended $3.2 billion on research and development in the Socioeconomic Objective of Health (a proxy for health and medical research).[1] The ABS data provides a similar picture for research by 'private not for profit' research organisations in the SEO of Health, which is predominantly undertaken by medical research institutes (MRIs); in 2014-15, nearly $197 million was expended by these organisations on research and development in the SEO of Health.[2] With around $3.4 billion spent every year on health and medical research in Australian universities and MRIs, the NHMRC funding of around $800 million per year represents around a quarter of the total.

Focusing just on Commonwealth competitive funding schemes, health and medical research is funded by Cancer Australia and the Australian Research Council in addition to the National Health and Medical Research Council (NHMRC). More information about funding sources is provided at Appendix 1.

Turning specifically to the NHMRC, its funding model does currently enable the funding of research into brain cancers and other low survival rate cancers. In 2016, for example, the funding announced by the NHMRC included research into brain lymphoma, the development of a device to improve brain cancer imaging, a clinical trial of a therapy for glioma, the trial of

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[1] Australian Bureau of Statistics 81110DO006_2014 Research and Experimental Development, Higher Education Organisations, Australia, 2014, Table 1.1

[2] Australian Bureau of Statistics 81090DO007_201415 Research and Experimental Development, Government and Private Non-Profit Organisations, Australia, 2014-15, Table 1.1

a drug for the treatment of glioblastoma in elderly patients, and the trial of a therapy for pediatric neuroblastoma.[3] All of these relate to brain cancer of one form or another.

In relation to the assertion the NHMRC funding model favours funding for types of cancer that attract more non-government funding, there is nothing explicitly requiring this; for example, there is no requirement for NHMRC funding to leverage funding from other sources. There are, however, some factors that influence the diseases that researchers choose to investigate and the applications they make for NHMRC funding. Some of these have implications for research into brain cancers and other low incidence cancers.

Funding the indirect costs of research

The NHMRC typically only funds the direct costs of research, leaving the organisation undertaking the research to meet the indirect research costs from other sources. Indirect costs include facilities maintenance (rent, electricity, heating, air-conditioning, cleaning, waste removal, facilities management, etc), and administration costs (salaries of administrative staff, IT support, business development offices, financial management, human resources and OH&S).

There are some Commonwealth Government subsidies for indirect research costs. Like the NHMRC, the existing Australian government schemes which provide support for the indirect research costs at universities and medical research institutes do not discriminate against cancer research. But while they don’t discriminate, they also do not fund all the indirect research costs. The 2013 report of the Strategic Review of Health and Medical Research (the McKeon Review) found that Australian Government support for indirect research costs was at the rate of 30% for universities and 20% for medical research institutes, and recommended that the rate be increased to 60%.[4] This has not occurred. As a consequence of the continuing under funding of indirect research costs, researchers need to find other sources of funding for the balance of the indirect costs. In the case of universities and medical research institutes, these sources include their own funds and philanthropic funding; some of the latter are directed towards supporting research into specific diseases.

The availability of funding from philanthropic sources to meet the indirect costs of research can influence the types of research that an organisation will undertake and the applications that it will make to the NHMRC for funding. To the extent that there is more funding available from non government sources to support research into a particular disease, this can lead to more applications to the NHMRC for funding in that area. This can favour research into areas that have strong philanthropic support. Conversely, areas of research that receive relatively less funding from non government sources can be less successful in the open, competitive grant schemes administered by the NHMRC and other government funding agencies.

Research Australia submits that to address the influence philanthropic and other non government funding can have on the allocation of Government funding through competitive grant programs, the Government should fully fund the indirect costs of research for which it provides competitive grant funding. In the case of universities, this requires an increase in funding provided through the Department of Education and Training via the Research Block Grant schemes.[5] In the case of MRIs, it requires additional funding

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[3] National Health and Medical Research Council, Results of the 2016 Grant Application Round, APP IDs 1120171; 1124160; 117501; 125204; 125036.

[4] Australian Government, Report of the Strategic Review of Health and Medical Research, 2013, Recommendation 10

[5] For further information, refer to https://www.education.gov.au/research-block-grants

to the NHMRC to enable it to increase the rate of funding provided to MRIs through the Independent Research Institute Infrastructure Support Scheme (IRIISS).

Research Australia acknowledges that NHMRC competitive grant funding has become even more competitive in recent years and that many research applications that are worthy of funding are not funded because the number of applications eligible to be funded well exceeds the available funding. In a 2015 discussion paper provided as part of a review of NHMRC funding programs, the NHMRC reported concerns among researchers that this had led to 'conservatism in the development and assessment of research proposals'.[6] This 'raising of the bar' for research applications has also favoured researchers with established track records and with initial research data that supports the research application.

These factors may to some extent work against diseases where the approaches being taken are novel and/or where there is little alternative funding or support for the initial research that can build the case for NHMRC funding. Full funding of the indirect costs of research by Government would allow more philanthropic funding to be directed to support novel early stage research and early career researchers, in turn helping to improve their chances of securing Australian Government competitive grant funding. Enabling more philanthropic funding to be directed to novel research areas could provide more opportunities for the funding of research into rare diseases, and help make funding applications for research into these areas more competitive.

b. the obstacles to running clinical trials for brain cancers and other cancers with relatively lower rates of incidence, with regard to:

i. funding models that could better support much-needed clinical trials, and

Many of the obstacles to running clinical trials for brain cancers and low incidence cancers are the same as those faced in running clinical trials generally, although often amplified in respect of low incidence cancers and rare diseases. These include a lack of data, a lack of access to data by researchers (leading to difficulties with recruitment), and a lack of capacity in our health system and research communities to effectively and efficiently support clinical trials. There are several initiatives underway at the Commonwealth, State and Territory levels to increase the number of clinical trials undertaken in Australia, improve access to Australian and international clinical trials for patients, and improve the conduct of clinical trials. For example, the priorities for funding from the Medical Research Future Fund include the following:

- Provide start-up investment in disease or therapy-focussed clinical registries supported by a national framework to maximise interoperability and value of research to clinical practice.
- Provide infrastructure support for existing and new national clinical trial networks to enhance innovation, collaboration, clinical research capacity and patient participation.

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[6] NHMRC, Structural Review of NHMRC's Grant Program, July 2016 p.5

- Invest in extension of clinical trials of proven therapies with limited opportunity for further commercial sponsorship to at-risk groups including adolescents and young adults, culturally diverse groups, and people with complex co-morbidities.[7]

While not specifically targeting brain cancers and other low incidence cancers these initiatives will be beneficial for these cancers (and for rare diseases more generally).

Research Australia submits that existing initiatives to improve clinical trials should be supported and their success in improving access to, and the conduct, of clinical trials for: a) all diseases; and b) brain cancers and other low incidence cancers, should be evaluated before any specific funding models are developed for clinical trials for brain cancers and other low incidence cancers.

b. the obstacles to running clinical trials for brain cancers and other cancers with relatively lower rates of incidence, with regard to:

ii. funding support for campaigns designed to raise awareness of the need for further research, including clinical trials;

Increasing awareness of the need for further research is an important role for patient advocacy groups and usually goes hand in hand with their efforts to raise funds from sources other than government. The second part of this term of reference is inquiring into *'funding support for campaigns designed to raise awareness of the need for further research funding'*. While it is included in the term of reference relating to the obstacles to clinical trials it does not appear to be limited to raising awareness for funding for clinical trial research.

Research Australia assumes the purpose of the proposed awareness raising campaigns would be to encourage individuals to donate to research (rather than to put pressure on the Government to provide research funding).

Research Australia submits if the aim is to support fundraising from the public, funding for these awareness campaigns should remain the responsibility of the patient groups. It would be more efficient and effective for the Commonwealth to allocate already stretched funding directly to more health and medical research.

Research Australia further submits using Commonwealth funds to increase public giving is through research funding programs that match Government funding with funds raised from the public. Cancer Australia's Priority Driven Collaborative Cancer Research Scheme is the best Australian example of this approach.[8]

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[7] Australian Government, Australian Medical Research and Innovation Priorities 2016-2018

[8] https://canceraustralia.gov.au/research-data/research/priority-driven-research/

c. The low survival rate for brain cancers, lack of significant improvement in survival rates, and strategies that could be implemented to improve survival rates;

Research Australia acknowledges that, sadly, brain cancers have relatively low survival rates and that there has been little improvement in five-year survival rates in recent decades. Unfortunately, this is not just true of brain cancers. Data from the Australian Institute of Health and Welfare lists brain cancer as having only a 1.2% improvement in survival rates between 1984-88 and 2009-2013. In the same period survival rates for cancers of the bladder, larynx and lip have declined and mesothelioma has shown no change. Other cancers with low rates of improvement include melanoma of the skin, cervix, pancreas and ‘other digestive organs’.[9]

Ideally, all cancers with low rates of improvement in survival would receive more funding. How to allocate limited funding between different cancers, and indeed between all types of diseases is an essential consideration for governments when developing strategies to support research in specific disease areas or groups.

Internationally, there are strategies to address research into low incidence cancers, such as the International Rare Cancers Initiative (IRCI). Established in 2011, IRCI aims to address the relatively inferior average outcomes for patients with rare cancers compared to cancers generally.[10]

Like low incidence cancers, rare non-cancer diseases are also often associated with relatively poor survival rates, limited improvements in survival over time, and limited funding opportunities. To address this issue, many countries have developed Rare disease policies as a means of providing a framework for a considered and comprehensive approach to the provision of research, diagnosis and access to treatment for rare diseases, including low incidence cancers. In such cases, where the only available therapies are often experimental, an approach that brings research, diagnosis and treatment together in the one policy is particularly valuable.

Health and medical research is undertaken around the world and improvements in survival are related to global research rather than specifically to the volume or subject of Australian research. The implications of this for a rare disease policy include considering the relative need for research on a global scale; identifying areas where Australia has specific expertise and can best contribute to the global research effort; and the opportunity to promote and support international collaborations. The latter can play a significant role in providing the opportunity for Australian patients with rare diseases to participate in clinical trials, which are often the only hope for an effective treatment.

Research Australia encourages consideration of the development of an appropriate policy framework for rare diseases as a means of addressing the Inquiry’s Terms of Reference.

In addition to providing a clear rationale for research funding, such a policy provides the opportunity for long term funding stability and transparency about the level of funding

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[9] Australian Institute of Health and Welfare 2017. Cancer in Australia 2017. Cancer series no.101. Cat. no. CAN 100. Canberra: AIHW. Figure 5.5 and online table

[10] http://www.irci.info

available and the eligibility criteria. This helps support the development of research in low survival rate/ rare disease areas in a sustainable manner and encourages engagement with patient groups and the health system. It also helps to depoliticise the decisions about how and where funding is allocated. **Research Australia submits that new funding would be required to support the provision of diagnosis services, specialised health care and research if such a policy was to be successful.**

There are also Rare Disease policies in 22 countries in Europe, and the USA. A few examples of Rare Disease Policies from around the world are summarised in Appendix 2.

d. Other relevant matters

The Terms of Reference raise a number of definitional and other issues which complicate what might on the surface appear to be a relatively simple question of determining whether, and how much, additional funding should be provided for research into cancers with low survival rates.

The nature of brain cancers

There are more than 40 recognised different brain cancers, with different causes and prognoses[11]. Recent shifts in our understanding of cancer at the molecular level has led to cancers being viewed and treated at the mutation specific level rather than by the site of origin of the cancer. Some have more in common with cancers in other parts of the body than they do with other brain cancers.

So, research that leads to a cure for one type of brain cancer will not benefit all people with brain cancer. Conversely, research into cancers that occur in other parts of the body can lead to advances for some brain cancers.

The role of non-disease specific research

It is worth noting not all research is disease specific; and yet this non-disease specific research often makes enormous contributions to the treatment of diseases.

For example, understanding how to regulate the immune system to trigger certain immune responses and inhibit others is leading to treatments for a broad range of different diseases including conditions as varied as diabetes and cancer. This basic research, which underpins research into treatments for specific conditions, may lead to treatments for brain cancer but is not brain cancer research per se.

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[11] Cancer Council Australia, http://www.cancer.org.au/about-cancer/types-of-cancer/brain-cancer.html

Allocating limited funding for health and medical research among competing priorities

There are many philosophical and ethical questions raised by the dilemma of how to allocate the limited available funding for health and medical research among competing priorities. Some of these are:

- Should every type of brain cancer (and presumably every other disease) receive the same funding on the basis that all disease causes harm and one person's suffering is as important as another's, regardless of how many people suffer from a particular disease?
- Should we allocate funds to diseases based on how common they are in the community, so that diseases with the highest prevalence get the most money?
- Should we allocate money to diseases with the highest mortality rates?
- How do we differentiate between a disease that affects older people and one that affects younger people?
- What weighting do we give to the quality of life of patients?
- What priority should be given to the likelihood of success of the research
- What priority should be given to the quality of the research application?
- What priority should be given to the capability of the researcher?

Consideration of a national framework that enables thinking and ultimately action around some of the se complex issues could provide a pathway addressing the many issues related to a rational and equitable distribution of research funding for brain cancers, low incidence cancers, cancers with low survival rates and other rare diseases.

Conclusion

Research Australia acknowledges that determining the appropriate allocation of funding to research into a particular disease is not easy.

Policymakers and those of us seeking to influence policy need an informed and nuanced understanding of the nature of health and medical research if we are to make the most effective use of Australia’s research expertise. This includes providing a clear rationale for research funding, long term funding stability, transparency about the level of funding available and the eligibility criteria.

We should ask the question from time to time about whether the allocation of funding to a particular disease or strategy is appropriate relative to other competing demands for funding. This approach ensures a considered and effective response, with the best possible outcomes for Australia.

Research Australia is pleased to provide any further information the Committee may require.

Appendix 1 Australian funding for HMR

The Government's $9.7 billion annual investment in research and development (R&D) is fragmented across 15 portfolios. Funding for health and medical research (HMR) derives from a number of these portfolios and from various programs; some are HMR specific while others are more general.

The most readily identifiable Australian Government funding for HMR comes from the Department of Health and the Department of Education and Training. The bulk of this flows to universities and MRIs, and it makes up more than one third of all expenditure in Australia on health and medical research and development.

In 2015-16, the NHMRC is expected to provide $846 million in funding for health and medical research.

The share of University Research Block Grant Funding related to HMR conducted by universities is the next largest component, at around $679 million. From there the amounts get significantly smaller, with a contribution of around $94 million from the Australian Research Council programs, $89 million in tax subsidies from the R&D Tax Incentive, $58 million in other support for research provided by the Department of Health and around $35 million in health related work by the CSIRO.[12] There are other elements of funding which are not included because the HMR related component cannot be specifically identified from available data. For example, the Department of Industry, Science and Innovation provides a range of programs that support R&D by Australian businesses, including those developing health related products and services.

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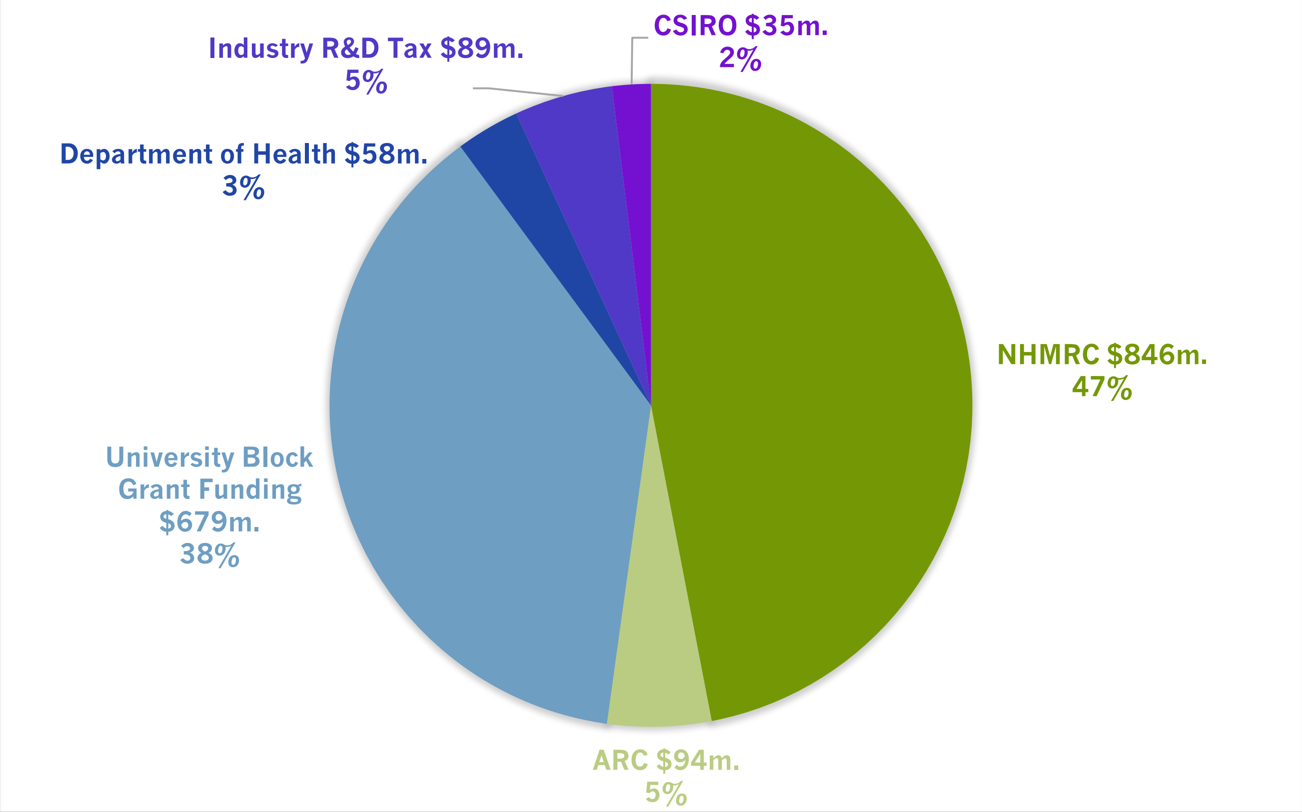
[12] The source for the data is the Science, Research and Innovation (SRI) Budget Tables 2015-16 for the SEO of health. The Block Grant Funding is estimated to be 34% of the value of university block grants, based on ABS data showing that 34% of all Higher Education R&D is conducted in the SEO of health. The Australian Government's 2015-16 Science, Research and Innovation Budget Tables; Analysis of ABS data on Higher Education R&D expenditure by SEO reveals that the SEO of Health accounts for 34% of total higher education R&D expenditure. *Source: ABS 8111.0, Research and Experimental Development, Higher Education Organisations, Australia 2012, SEO Health ($3,270,969,000) divided by total expenditure ($9,609,736,000).* Another component of funding which is not included in this table because it cannot be accurately estimated is the portion of funding received by universities from the Commonwealth Government which is used to fund research activities, including HMR. The Australian Bureau of Statistics reported that in 2012, General University Funds, including revenue from the Australian Government (but not including the categories in the Pie Chart 1) was $5,340 million.

Pie Chart 1. Funding of HMR and medical innovation by the Australian Government Budget Estimates 2015-16[13]



The components identified in the above chart are explored below.

NHMRC Funding

The NHMRC is responsible for the operation of the Medical Research Endowment Account (MREA). The MREA receives allocations from the Australian Government as part of the Budget process and disbursements are made in instalments to grant recipients, usually over several years. The NHMRC provides funding programs for medical research and public health research; and research training.

The NHMRC provides a number of different fellowship schemes, and these tend to combine elements of further training and the provision of support to undertake research, blurring the lines between support for research and for research training. The total value of grants allocated for fellowships and other ‘people support’ in 2015 was $172 million; approximately 22% of the value of all NHMRC grants.[14]

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[13] Ibid

[14] [14] Australian Government, National Health and Medical Research Council, *2015 Outcomes by Scheme, Table 2*, http://www.nhmrc.gov.au/grants-funding/outcomes-funding-rounds, accessed 27 April 2016

Medical Research Future Fund

Legislated in 2015, the Medical Research Future Fund is to provide funding for medical research and medical innovation. An endowment fund targeted to have $20 billion in capital by 2020/21, the first funding of approximately $60 million is to be provided in 2016/17. This amount is forecast to grow year by year as the capital increases, reaching $1 billion per annum.

Australian Research Council funding

The Australian Research Council (ARC) provides funding for research and research training to higher education institutions. Grants awarded for commencement in 2014-15 total $663 million.[15] While the ARC does not fund 'medical and dental research' as defined by its own funding rules, it funds a number of research areas which fall within a broad definition of health and medical research or are closely related to it, including bioengineering and research into the basic understanding of biological processes in humans and different stages of human development.[16] In 2015-16, ARC expenditure on health and medical research is estimated to be $94 million.[17]

University Block Grant funding

There are six Commonwealth Government grants programs that support research activities in the higher education sector. Funding for these programs is allocated based on criteria related to the conduct of research, such as the value of an institution's competitive research grants and the number of research students. In 2016, expenditure on these programs is expected to be $1.81 billion.[18]

Department of Health

In addition to funding for the NHMRC, the Department of Health funds a number of programs directly from its own budget; examples are the Cancer Clinical Trials, longitudinal studies of Men's and Women's Health, Drug and Alcohol Research and the National Acoustic Laboratories. In 2015-16, the estimated expenditure on these programs is $58.2 million.[19]

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[15] Australian Research Council, *Annual Report 2014-15*, Chapters 4 and 5.

[16] Refer to the ARC Medical Research Policy, effective 24 August 2015 at http://www.arc.gov.au/arc-medical-research-policy

[17]The Australian Government's 2015-16 Science, Research and Innovation Budget Tables,

[18] Dept. of Education and Training website, 4 February 2016 at https://www.education.gov.au/research-block-grants www.innovation.gov.au/Research/ResearchBlockGrants/Pages/default.aspx

[19] The Australian Government's 2015-16 Science, Research and Innovation Budget Tables, Table 4, Department of Health and Ageing (excluding NHMRC)

Industry R&D Tax Incentive

The Australian Government provides the R&D Tax Incentive to support companies undertaking research and development by providing additional tax incentives. In 2015-16 the total value of the R&D Tax Incentive is estimated to be $2.9 billion, of which $89 million relates to health R&D.[20]

Commonwealth Scientific and Industrial Research Organisation (CSIRO)

The CSIRO undertakes research across a wide range of areas, including health. Within health it covers a diverse range of fields from nutrition to the application of digital technologies. In 2015-16, CSIRO is estimated to spend $35 million on health and medical research.[21]

Other measures

The Australian Government provides a range of other programs to support research and/or commercial R&D, in particular from the Department of Industry, Science and Innovation. While some of the funding for these programs supports HMR and/or medical innovation, it is not possible to accurately quantify these amount and they have not been included in Pie Chart 1.

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[20] The Australian Government's 2015-16 Science, Research and Innovation Budget Tables, SEO of Health

[21] The Australian Government's 2015-16 Science, Research and Innovation Budget Tables, SEO of Health

Appendix 2 Rare Disease Policies

European action on rare disease

The European Commission has a policy on rare diseases. Any disease affecting fewer than 5 people in 10 000 is considered rare, and it has estimated that 5-8000 distinct rare diseases affect 6-8% of the EU population – between 27 and 36 million people.[22]

The EU policy seeks to pool scarce resources that are currently fragmented across individual EU countries on the basis that joint action helps patients and professionals share expertise and information across borders. Specific measures include:

- improving recognition and visibility of rare diseases
- ensuring that rare diseases are adequately coded and traceable in all health information systems
- supporting national plans for rare diseases in EU member countries
- strengthening European-level cooperation and coordination
- creating European reference networks linking centres of expertise and professionals in different countries to share knowledge and identify where patients should go when expertise is unavailable in their home country
- encouraging more research into rare diseases
- evaluating current screening population practices
- supporting rare diseases registries and providing a European Platform for rare diseases registration.
- Patient organisations are considered to be particularly important because they provide additional incentives for developing orphan drugs to combat rare diseases.[23]

The approach relies on individual countries to take action. 22 countries in Europe have developed national rare disease plans or strategies:

Austria
Belgium
Bulgaria
Croatia
Cyprus
Czech Republic
France
Germany
Greece
Hungary
Ireland
Italy
Latvia
Lithuania
Luxembourg
Portugal
Romania
Slovak Republik
Slovenia
Spain
The Netherlands
United Kingdom

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[22] http://ec.europa.eu/health/rare_diseases/policy/index_en.htm
[23] Ibid

EU funding for rare disease research

The EU has established an 80 billion Euro program to promote innovation and maintain European competitiveness, called Horizon 2020. As part of this program the EU is allocating funding towards rare disease research. To date, almost 900 million Euro, has been provided from Horizon 2020 and the EU predecessor Program FP7 to more than 160 collaborative projects related to rare diseases.[24] This funding is being coordinated through the International Rare Diseases Research Consortium (IRDiRC). (See below)

EURORDIS

EURORDIS is a non-governmental patient-driven alliance of patient organisations and individuals active in the field of rare diseases, dedicated to improving the quality of life of all people living with rare diseases in Europe. It is responsible for co-ordinating International Rare Diseases Day. [25]

US action on rare disease

The Office of Rare Diseases Research (ORDR)

The ORDR was established in 1993 within the Office of the Director of the National Institutes of Health (NIH). In 2011 ORDR was integrated into the newly established National Center for Advancing Translational Sciences (NCATS). As an integral part of NCATS, ORDR coordinates and supports rare diseases research, responds to research opportunities for rare diseases, and provides information on rare diseases.

The goals of ORDR are to identify, stimulate, coordinate and support research to respond to the needs of patients with rare diseases. To leverage its resources, ORDR fosters collaboration nationally and internationally.[26]

The ORDR:

- Recommends an agenda for conducting rare diseases research.
- Supports research and training of NIH rare diseases investigators, together with NIH Institutes and Centers, at the NIH Clinical Center hospital and medical research centers throughout the Nation.
- Supports a rare diseases clinical research network (http://www.rarediseasesnetwork.org/) and other scientific opportunities.
- Coordinates and encourages cooperation in rare diseases research.
- Responds to scientific opportunities and builds international collaborations.
- Supports an extensive scientific conferences program.
- Cosponsors, with the National Human Genome Research Institute, the Genetic and Rare Diseases Information Center.
- Provides information on genetic and acquired rare diseases in English and Spanish to patients, their families, health care professionals, and researchers.
- Compiles yearly reports for Congress and the public about NIH-supported scientific research plans on rare diseases.

National Organisation for Rare Disorders

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[24] https://ec.europa.eu/research/health/index.cfm?pg=area&areaname=rare

[25] http://www.eurordis.org

[26] https://rarediseases.info.nih.gov/asp/resources/ord_brochure.html

A non government organisation, the National Organization for Rare Disorders (NORD) provides advocacy, education and other services to improve the lives of all people in the USA affected by rare diseases.[27]

Rare disease in Australia

Australian Government

The Australian Government does not have a rare disease strategy or policy. The only Government programs specifically addressing rare diseases are the TGA Orphan Drugs registration program referred to above and the Life Saving Drugs Program, which provides funding for drugs to treat rare life threatening conditions where no suitable drug therapy is available through the Medical Benefits Scheme.[28]

The Department of Health and Ageing does provide funding for some other relevant programs, such as the Australian Pediatric Surveillance Unit's surveillance program for uncommon rare childhood diseases, complications of common diseases or adverse effects of treatment.[29]

COAG

The Australian Health Ministers Advisory Council (AHMAC) comprises the CEOs of the Commonwealth and State health departments, and its function is to advise the COAG Standing Committee on Health (the Health Ministers). In 2011 the AHMAC requested the preparation of a National Rare Diseases Scoping Plan. The scoping paper was prepared by the WA Health Department and presented to AHMAC in September 2013.[30] The paper recommended the creation of a National Rare Diseases Plan. The Plan as proposed would include education, surveillance, engagement of consumer groups, coordination of care utilising evidence based best practice, and the facilitation of research.

The AHMAC did not support the recommendation.[31]

Western Australia

The Government of Western Australia has a Rare Diseases Strategic Framework.[32]

Rare Voices

Rare Voices Australia (RVA) is a national, not-for-profit organisation established in 2012 with a vision to be 'the unified voice for ALL Australians living with a rare disease'.

RVA is Australia's national alliance advocating for those who live with a rare disease. RVA provides a strong common voice to promote health policy and a healthcare system that works for those with rare diseases.

RVA works with governments, researchers, clinicians and industry to promote research, diagnosis, treatment and services for all rare diseases in Australia. RVA are the unified voice of 1.2 million plus people affected by rare diseases throughout Australia (6–8% of the population), and 70 million globally. [33]

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[27] https://www.rarediseases.org

[28] http://www.health.gov.au/internet/main/publishing.nsf/Content/lsdp-criteria

[29] http://www.apsu.org.au

[30] The paper is available at https://www.yumpu.com/en/document/view/39801026/scoping-paper-on-the-need-for-a-national-rare-diseases-plan-for-

[31] http://health.thewest.com.au/news/1349/rare-diseases-find-common-ground

[32] http://ww2.health.wa.gov.au/Reports-and-publications/WA-Rare-Diseases-Strategic-Framework-2015-2018

[33] http://www.rarevoices.org.au

International action

International Rare Diseases Research Consortium (IRDiRC)

Launched in April 2011, the Consortium started out as a joint project of the European Commission and the National Institutes of Health in the United States of America, but since then other countries have become involved, including Canada and Japan. Australia's WA Department of Health is listed on the website as an IRDiRC member.

The IRDiRC aims to bring together regulatory agencies, researchers, patient group representatives, members of the biopharmaceutical industry and health professionals, and among the objectives that it has publicly set is the generation of 200 new therapies for rare diseases and diagnostic tools for most rare diseases by 2020.[34] Funding is open to researchers from around the world.

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[34] http://www.irdirc.org

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