Chapter 2

Funding for research into low survival rate cancers

2.1 The impact of effective research investment is clearly demonstrated by the increased survival rates for people with certain cancers, such as breast and prostate cancer.[1] Funding for cancer research comes from various sources, including the National Health and Medical Research Council (NHMRC), which, as discussed further below, recently restructured its grants program.[2]

2.2 This chapter commences by defining cancer research and then examines the various sources of funding for such research, focussing specifically on government funding through the NHMRC, Cancer Australia and the newly established Medical Research Future Fund (MRFF), as well as philanthropic and pharmaceutical funding.

2.3 The chapter then provides some context to the challenges facing funding for research into low survival rate (LSR) cancers by providing an overview of the Therapeutic Goods Administration (TGA), the Pharmaceutical Benefits Advisory Committee (PBAC) and the Pharmaceutical Benefits Scheme (PBS). The chapter concludes by examining the available funding for LSR cancers.

Cancer research

2.4 In 2016, Cancer Australia published a report into the funding for cancer research projects in Australia from 2016–2018, using data from grants awarded to these projects to the end of July 2015.[3]

2.5 This report identified that the Australian government is currently funding 74 per cent, or $187 million, of the $252 million that has been provided to 589 individual research projects for the period 2016–2018.[4] Ninety five per cent of these research projects are funded by a single source.[5]

2.6 The following figure illustrates how cancer research funding for this period has been allocated by reference to the Common Scientific Outline (CSO), a system

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1 See, for example, UNSW Sydney & SPHERE, *Submission 48*, p. 3; Ovarian Cancer Australia, *Submission 242*, p. 4; Professor David Walker, *Submission 269*, pp 2–3.

2 See, The Hon. Greg MP, 'Medical research reforms to improve our future health, *Media Release*, 25 May 2017.

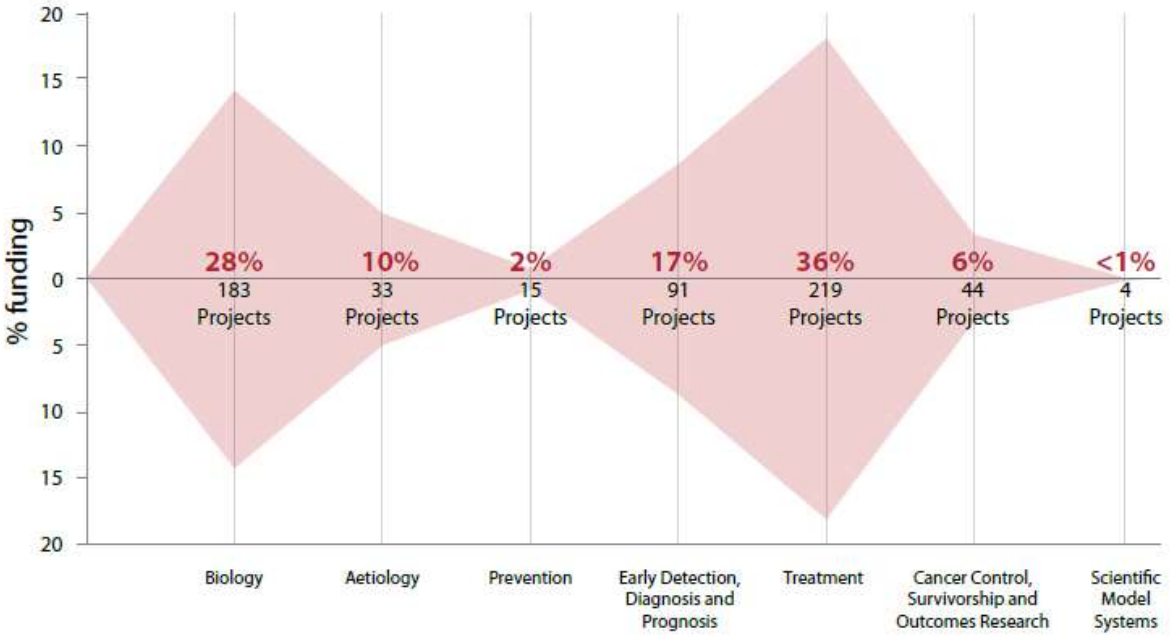
3 Cancer Australia, *Cancer Research in Australia 2016 to 2018 - Opportunities for strategic research investment – Highlights*, 2016.

4 Cancer Australia, *Cancer Research in Australia 2016 to 2018 - Opportunities for strategic research investment – Highlights*, 2016, p. 1.

5 Cancer Australia, *Cancer Research in Australia 2016 to 2018 - Opportunities for strategic research investment – Highlights*, 2016, p. 1.

which 'uses easily applied terminology to describe and classify research by where it best fits into the cancer research continuum'.[6]

Figure 1: The national pattern of cancer research funding in 2016 to 2018[7]



2.7 As can be seen in Figure 2, Cancer Australia also classified the cancer research funding during this period by reference to a system developed by the United States (US) National Cancer Institute, which is used to identify translational elements within CSO sub-categories.[8] These categories are defined as follows:

- Not Translational – basic research;
- Translational/Early – the translational process that follows fundamental discovery and precedes definitive, late-stage trials;
- Translational/Clinical – research at the clinical application end of the translational spectrum;
- Translational/General – research where difficulty in separating early and late translation/clinical research;
- Translational/Patient-oriented – research focussed on needs in the area of patient care and survivorship[9]

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6 Cancer Australia, *Cancer Research in Australia 2016 to 2018 - Opportunities for strategic research investment – Highlights*, 2016, p. 2.

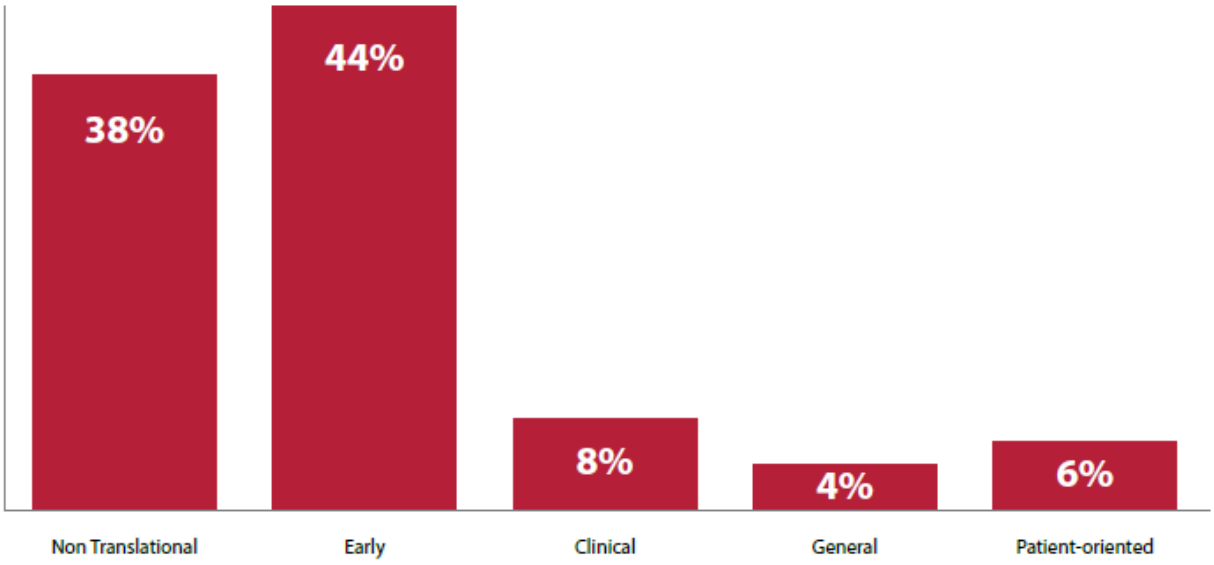
7 Cancer Australia, *Cancer Research in Australia 2016 to 2018 - Opportunities for strategic research investment – Highlights*, 2016, p. 2.

8 Cancer Australia, *Cancer Research in Australia 2016 to 2018 - Opportunities for strategic research investment – Highlights*, 2016, p. 3. This classification system is also used by the International Research Partnership.

9 Cancer Australia, *Cancer Research in Australia 2016 to 2018 - Opportunities for strategic research investment – Highlights*, 2016, p. 3.

2.8 This figure illustrates that translational research in the clinical, general and patient-oriented categories will receive less than 10 per cent funding each for the 2016–2018 period.

Figure 2: Percentage of funding to cancer research projects and programs classified by translation categories[10]



2.9 The lack of funding for the clinical stage of research was discussed by the Low Cancer Survivals Alliance (LCSA), which submitted that '[t]here is a lack of leadership by state and federal governments to encourage health services to support clinical trial research':

> Funding bodies such as the NHMRC traditionally do not support translational research, therefore these breakthroughs are often not capitalised on and further developed. Often funding for basic research is preferred over clinical trials, as it can have more immediate results. As an example, in February 2017 an incredible breakthrough was published in the international journal Nature for the genome sequencing of pancreatic nets, led by Melbourne University researchers. This research now needs to be supported and built upon, in order for it to have an impact on patient outcomes.[11]

2.10 Indeed, The Unicorn Foundation similarly identified that 'the current NHMRC model does not actively support translational research in low survival cancers' and advocated for 'a new model of funding' for the NHMRC and support for clinical trials for LSR cancers.[12]

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10 Cancer Australia, *Cancer Research in Australia 2016 to 2018 - Opportunities for strategic research investment – Highlights*, 2016, p. 3.

11 Low Survival Cancers Alliance (LSCA), *Submission 90*, p. 2.

12 The Unicorn Foundation, *Submission 101*, p. 4.

2.11 However, in her evidence to the committee, Professor Anne Kelso of the NHMRC made clear that her organisation funds discovery through to translational research:

> The NHMRC is interested in funding research that covers the whole spectrum from discovery research, which might help us to understand the origins of disease and also the origins of health, but we seek to fund across the full spectrum from discovery through to translation into better health care. We fund many clinical trials that assist in that translation of new ideas, new discoveries, into better health care. We also fund research to improve health services across the board. So there is a very broad range of research that NHMRC funds, and some of it is very directly translational and some of it is earlier stage.[13]

2.12 Despite this, Professor Stephen Fox identified a 'tension between true translational clinical work and some of the basic discovery work', suggesting how funding of clinical trials could jeopardise funding of discovery research:

> There are the basic NHMRC studies, which are very much discovery-type stuff, and then there is the other end of the spectrum, which is the clinical trials-type activity. I think the clinical trials activity is usually fairly explicit and straightforward in what the aims are. I think there is an understanding behind that. The issue is that running a clinical trial, as I am sure you have heard, is an incredibly expensive endeavour and takes a large slice of the budget. So you only have to, I suppose, fund a few of those and you have basically taken a huge chunk of your budget away from the discovery sector.[14]

2.13 Advocating for a balance between discovery and translational research funding, Professor Manuel Graeber identified that currently, 'there is no balance' and further, that:

> …translational outcomes, to some extent, represent marketing speak. Politicians must be aware of the power they have. If the decision is made to favour an area then everybody, in the current funding climate, will jump at this. Administrators will and researchers have to follow but that is wrong. Researchers are the ones that are supposed to come up with the innovations. They are not being listened to often nowadays, because of the way—based on a global trend—science has changed. In the old days it was just idealists working somewhere without pay—some still work without pay today. Generally institutions cannot afford it and that is the big problem—the research dollars. I cost the university money. Teaching is much more attractive, but, of course, it would be living on intellectual credit if we would not support the research. That is the future.

13 Professor Anne Kelso, Chief Executive Officer (CEO), National Health and Medical Research Council (NHMRC), *Committee Hansard,* 19 May 2017, p. 30.

14 Professor Stephen Fox, Director of Pathology, Peter MacCallum Cancer Centre, *Committee Hansard*, 4 August 2017, p. 32.

> I think it is really important how this is marketed—directed by the politicians. Translational outcomes flies well with politicians, but it is important to really look at the substance. What is really being produced? Where is innovation coming from? How can we enable that? It will not come just through some policy decisions. Scientists are not motivated to engage in it, because it is like the 'fashion scientist', who makes a career by being in policy making. We are about innovation. We are supposed to find new things that are reproducible. That is our job. It is not to compete with politicians implementing policies. That is my personal view, so do not blame it on the university. That is my view, and I am happy to defend it.[15]

2.14 In its report, Cancer Australia concluded by identifying the following opportunities for future strategic investment in cancer research, some of which will be addressed in chapter 5 of this report:

- targeted research investment by tumour site;
- targeted research investment by research category;
- translational research; and
- research collaborations.[16]

Sources of funding

2.15 There are many different government and non-government sources of funding for medical research. Although government funding can include funding directly from the Department of Health (DoH), this chapter exclusively examines funding from the NHMRC, Cancer Australia and the MRFF, which were the government sources most frequently referred to in submissions and evidence to the committee. At points throughout this report, there may be references to other sources of government funding.

2.16 In addition to government funding for medical research, a significant amount of funding is also provided by non-government sources, particularly philanthropic and pharmaceutical sources. For this reason, this section also briefly examines these sources of funding.

The National Health and Medical Research Council

2.17 The function of the NHMRC, a statutory body which operates pursuant to the *National Health and Medical Research Council Act 1992* (NHMRC Act), is to assist the Chief Executive Officer (CEO) of the NHMRC, a position currently held by Professor Kelso, in the performance of her functions.[17] These functions are:

> (a) in the name of the NHMRC, to inquire into, issue guidelines on, and advise the community on, matters relating to:

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15 Professor Manuel Graeber, Barnet-Cropper Chair of Brain Tumour Research, Brain and Mind Centre, University of Sydney, *Committee Hansard*, 18 May 2017, p. 63.

16 Cancer Australia, *Cancer Research in Australia 2016 to 2018 - Opportunities for strategic research investment – Highlights*, 2016, p. 4.

17 *National Health and Medical Research Council Act 1992*, s. 5C.

(i) the improvement of health; and

(ii) the prevention, diagnosis and treatment of disease; and

(iii) the provision of health care; and

(iv) public health research and medical research; and

(v) ethical issues relating to health; and

(b) to advise, and make recommendations to, the Commonwealth, the States and the Territories on the matters referred to in paragraph (a); and

(c) to make recommendations to the Minister on expenditure:

(i) on public health research and training; and

(ii) on medical research and training;

including recommendations on the application of the Account; and

(d) any other functions conferred on the CEO in writing by the Minister; and

(e) any other functions conferred on the CEO by this Act, the regulations or any other law; and

(f) any functions incidental to any of the foregoing.[18]

2.18 The minister may also delegate additional functions to the CEO.[19]

2.19 The Council of the NHMRC[20] provides advice to the CEO in relation to the performance of these functions, and also performs any other functions conferred by the minister, the NHMRC Act, its regulations, or any other law.[21]

2.20 Mr Greg Mullins of Research Australia observed that NHMRC funding has been 'effectively flatlining in recent years'[22] and spoke to the positive effects of adequately funding the NHMRC:

> One of the things that happened with NHMRC funding in the period from 2000 to about 2010 was that the funding was doubled, and then it was doubled again. That was a great outcome; it was really good news for the sector. What it has done is attract a whole lot more people into the field. We are seeing more people undertaking PhDs in this area. I think the latest budget figures were predicting that Australia-wide we were going to move from 9½ thousand PhD completions last year to 12½ thousand by 2019-20. So we are seeing an increasing number of people coming into this area.[23]

18 *National Health and Medical Research Council Act 1992*, ss. 7(1).

19 *National Health and Medical Research Council Act 1992*, s. 82.

20 Established pursuant to s. 20 of the *National Health and Medical Research Council Act 1992*.

21 *National Health and Medical Research Council Act 1992*, s. 21.

22 Mr Greg Mullins, Head of Policy, Research Australia, *Committee Hansard*, 7 June 2017, p. 43.

23 Mr Mullins, Research Australia, *Committee Hansard*, 7 June 2017, p. 46.

2.21 However, Mr Mullins opined that the availability of NHMRC funding to support these researchers and their work is lacking, which is consequently reflected 'in things like the drop in the success rates with NHMRC funding'.[24] The difficulty of securing NHMRC funding was also identified by Dr Bryan Day, who informed the committee that 'the competition in the current NHMRC funding pool is incredibly high, because the pot of money is small'.[25]

The NHMRC's previous approach to funding

2.22 The NHMRC is 'the largest single funder of health and medical research in Australia', covering 'the breadth of health and medical research needs'.[26] In its submission, the NHMRC set out the process by which it considers funding applications:

> Consistent with the NHMRC Act, NHMRC focuses on the relevance of research proposals for health, rather than defining 'health and medical research' as a set of research disciplines. NHMRC will fund research in any or all areas relevant to health. It will also accept grant applications in any research discipline and applicants are provided with an opportunity within their application to explain how their research will lead to improved outcomes in health.
>
> Most NHMRC funding is awarded in response to investigator-initiated applications in which the research is conceived and developed by the researchers. A smaller proportion of funding is directed to specific areas of unmet need, e.g., through Targeted Calls for Research, special Centres of Research Excellence, Partnership Centres and some Partnership Projects.
>
> The primary criterion for all funding decisions is excellence. NHMRC relies on review by independent experts to identify the best applications, based on the significance of the research, the quality and feasibility of the research proposal, and the track record of the investigators. Rigorous processes of expert review ensure transparency, probity and fairness.
>
> When applications for funding are received, the office of NHMRC manages the expert assessment of applications by independent experts. The outcomes of expert review are used to determine which applications will be recommended for funding. NHMRC's [Research Committee] recommends those applications to be funded through NHMRC Council to the CEO who submits them for approval to the Minister with portfolio responsibility for NHMRC.[27]

2.23 The NHMRC also outlined its capacity to direct funding to priorities, as required:

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24 Mr Mullins, Research Australia, *Committee Hansard*, 7 June 2017, p. 46.

25 Dr Bryan William Day, Team Head, Translational Brain Cancer Research Laboratory, QIMR Berghofer Medical Research Institute (QIMR Berghofer), *Committee Hansard*, 6 June 2017, p. 38.

26 NHMRC, *Submission 87*, p. 3.

27 NHMRC, *Submission 87*, p. 3 (citations omitted).

> NHMRC's range of funding schemes also provides the flexibility necessary for targeting research and capacity building in key areas of need in the health system. Each year NHMRC sets aside a component of the [Medical Research Endowment Account] to address identified priorities. Priorities are often implemented through additional funding provided for existing NHMRC schemes, such as the Centres of Research Excellence scheme.
>
> Each year, a small proportion of the total annual expenditure budget is set aside to fund priority research areas through its Targeted Calls for Research (TCR) funding program. A TCR is a specific funding mechanism that invites grant applications to address a specific health issue. NHMRC may initiate a TCR to address additional major issues that arise or in cases where substantial gaps in evidence are identified. The aim of a TCR is to stimulate or greatly advance research in a particular area of health and medical science that will benefit the health of Australians. Through the TCR program, NHMRC has an opportunity to identify and subsequently fund emerging health problems in Australia.[28]

2.24 In respect of cancer funding in particular, the NHMRC stated that it 'is the biggest funder of cancer research in Australia, accounting for 56% of all funding nationwide'.[29] The allocation of cancer research funding:

> …is based on the review of each grant against a range of investigator-provided data classifications including Burden of Disease allocations, fields of research, keywords, grant titles and media summaries. Many grants address more than one cancer type and in these cases the full value of each is attributed to each relevant cancer type.[30]

2.25 The following table sets out the NHMRC's funding for cancer research for the period 2012 to 2016, across all grant types, where the allocation of funding is:

> …based on the review of each individual grant against a range of investigator provided data classifications including Burden of Disease allocations, fields of research, keywords, grant titles and media summaries. Many grants address more than one cancer type and in these cases the full value of each is attributed to each relevant cancer type.[31]

28 NHMRC, *Submission 87*, p. 3.

29 NHMRC, *Submission 87*, p. 4 (citations omitted).

30 NHMRC, *Submission 87*, p. 4.

31 NHMRC, *Submission 87*, Attachment A, p. 7.

Table 1: NHMRC cancer research expenditure 2012 to 2016[32]

Cancer Type	2012	2013	2014	2015	2016	Total
Leukaemia	$23,803,468	$19,769,414	$24,096,017	$25,068,518	$23,704,073	$116,441,490
Breast Cancer	$24,803,186	$21,852,140	$20,508,426	$23,924,737	$21,469,127	$112,557,616
Colorectal Cancer	$17,110,467	$14,400,726	$11,047,089	$13,427,898	$12,371,421	$68,357,601
Childhood Cancer	$13,873,871	$12,425,114	$11,839,850	$12,219,439	$10,358,657	$60,716,931
Melanoma	$11,083,287	$11,012,931	$11,943,557	$13,145,930	$13,403,015	$60,588,720
Prostate Cancer	$15,714,971	$10,777,957	$8,299,874	$8,895,471	$8,458,090	$52,146,363
Hodgkin's Lymphoma	$10,448,532	$8,507,097	$8,081,885	$8,088,540	$6,100,138	$41,226,192
Ovarian Cancer	$11,516,436	$10,569,137	$7,690,016	$4,393,454	$4,701,048	$38,870,091
Brain Cancer	$7,973,145	$7,207,891	$8,341,513	$8,469,035	$6,630,739	$38,622,323
Lung Cancer	$5,822,566	$6,795,275	$7,610,659	$7,988,644	$7,769,633	$35,986,777
Pancreatic Cancer	$9,812,427	$8,923,906	$6,841,808	$3,653,131	$4,117,523	$33,348,795
Multiple Myeloma	$7,055,307	$6,079,353	$5,654,967	$5,851,116	$4,769,828	$29,410,571
Liver Cancer	$3,209,094	$3,812,146	$5,470,925	$5,275,872	$4,455,742	$22,223,779
Stomach Cancer	$3,731,366	$3,716,477	$2,662,717	$3,608,741	$4,695,318	$18,414,619
Mesothelioma	$1,914,182	$1,696,954	$2,097,639	$3,117,450	$2,142,460	$10,968,685
Bone Cancer	$2,515,135	$1,986,772	$2,202,010	$2,205,394	$1,383,337	$10,292,648
Oesophageal Cancer	$3,059,316	$2,667,775	$1,781,589	$1,524,016	$1,148,474	$10,181,170
Endometrial Cancer	$2,362,829	$2,039,453	$1,587,515	$1,474,190	$1,420,730	$8,884,717
Non-Hodgkin's Lymphoma	$1,488,384	$1,533,322	$2,166,269	$2,210,672	$1,433,272	$8,831,919
Head and Neck Cancers	$1,917,637	$1,929,367	$1,691,935	$1,195,252	$1,003,233	$7,737,424
Cervical Cancer	$1,131,369	$1,442,060	$1,909,510	$1,040,493	$1,308,283	$6,831,715
Testicular Cancer	$1,453,958	$1,602,101	$1,183,460	$1,194,662	$895,991	$6,330,172
Kidney Cancer	$1,340,442	$852,278	$667,439	$420,627	$321,571	$3,602,357
Bladder Cancer	$464,861	$467,727	$537,361	$304,437	$198,704	$1,973,090
Thyroid Cancer		$97,733	$428,827	$551,373	$535,646	$1,613,579
Vulvar Cancer	$439,249		$397,276	$383,721	$373,346	$1,593,592
Adrenal Cancer	$295,384	$250,452	$119,529	$165,361	$477,340	$1,308,066
Anal Cancer	$202,025	$132,337	$122,911	$60,173		$517,446
Eye Cancer	$188,285				$36,134	$224,419
Parathyroid Cancer				$124,531		$124,531
Pituitary Cancer		$17,949	$38,437	$13,335	$21,197	$90,918

2.26 The NHMRC also provided the following additional table comparing its research expenditure with incidence, mortality and survival rates, for 'all persons', except in the case of the following gender-specific cancers: cervical, ovarian, uterine,

32 NHMRC, *Submission 87*, Attachment A, p. 7.

prostate and testicular cancers.[33] The data for cancer incidence, mortality and survival rates were sourced from the Australian Institute for Health and Welfare (AIHW).[34]

Table 2: NHMRC cancer research expenditure comparison with incidence, mortality and survival rates[35]

Cancer Type	NHMRC Expenditure 2012 to 2016	2013 Age-standardised incidence rate	2014 Age-standardised 5 yr mortality rate	Five-year relative survival from selected cancers, 2009–2013 (%)
Leukaemia	$116,441,490	13.3	6.2	-
Breast Cancer	$112,557,616	63.6	10.5	90.2
Colorectal Cancer	$68,357,601	57.7	14.9	68.7
Melanoma	$60,588,720	50.3	5.5	90.4
Prostate Cancer	$52,146,363	151.3	25.8	94.5
Hodgkins Lymphoma	$41,226,192	2.6	0.4	87.5
Ovarian Cancer	$38,870,091	10.6	6.8	44.4
Brain Cancer	$38,622,323	6.5	5.3	22.1
Lung Cancer	$35,986,777	42.6	30.5	15.8
Pancreatic Cancer	$33,348,795	10.9	9.3	7.7
Multiple Myeloma	$29,410,571	6.3	3.3	48.5
Liver Cancer	$22,223,779	6.9	6.4	17.3
Stomach Cancer	$18,414,619	8.1	4.2	28.5
Uterine Cancer	$12,351,703	18.6	3.4	83.2
Mesothelioma	$10,968,685	2.7	2.6	5.8
Bone Cancer	$10,292,648	0.8	0.4	69.7
Oesophageal Cancer	$10,181,170	5.4	4.4	20.1
Non-Hodgkins Lymphoma	$8,831,919	19.4	5.5	74.3
Head and Neck Cancers	$7,737,424	17.2	3.8	-
Cervical Cancer	$6,831,715	6.8	1.7	72.1
Testicular Cancer	$6,330,172	6.4	0.2	97.9
Kidney Cancer	$3,602,357	11.9	3.4	74.9
Bladder Cancer	$1,973,090	9.7	3.7	53.3
Thyroid Cancer	$1,613,579	10.6	0.5	96.1
Anal Cancer	$517,446	1.5	0.4	67.1

Criticisms of the previous approach with respect to funding research into LSR cancers

2.27 A number of submitters and witnesses criticised the former NHMRC funding model—in place up until the minister's announcement on 25 May 2017—and its 'one size fits all' approach[36] asserting that it disadvantages,[37] or is biased against,[38] researchers into LSR cancers.

33 NHMRC, *Submission 87*, Attachment A, p. 8.

34 NHMRC, *Submission 87*, Attachment A, p. 8. See: Australian Institute for Health and Welfare (AIHW), *Cancer in Australia 2017*, Cancer series no. 101, 2017, Appendix B, pp 149–151. Figures for leukaemia were not from another AIHW report.

35 NHMRC, *Submission 87*, Attachment A, p. 8.

36 See, for example, Pancare Foundation, *Submission 9*, p. 2; Love for Lachie, *Submission 120*, p. 2.

2.28 For example, the Children's Cancer Research Unit (CCRU) of The Children's Hospital at Westmead outlined some issues that arise with respect to receiving NHMRC grants for research into LSR cancers:

> We believe that characteristics of low survival rate cancers can make it more difficult for associated research grant proposals to be considered "well designed (or to have) a near flawless design". The fact that a particular cancer is characterised by poor survival rates can reflect a more limited research base, leading to less scientific knowledge. This can mean a greater need for more open-ended research grant applications seeking to (for example) identify treatment targets, or biomarkers of response. However, these more open-ended proposals can be viewed by grant review committees and reviewers as "fishing expeditions" that may be less likely to be considered to have "objectives that are well-defined, highly coherent and strongly developed (and be either) well designed (or have) a near flawless design". Similarly, low survival rate cancers may have fewer experimental models (cell lines, mouse and other animal models) available for study. It can also be challenging to access statistically informative and representative sample cohorts, or patient cohorts for clinical trials. Reduced resources for research could therefore also lead to reduced "scientific quality" and "significance and innovation" scores for NHMRC project grant applications, as well as negatively impacting the team's "track record". One of the most problematic issues is how the determination of "an issue of great importance to human health" is made, as this judgement can clearly be made according to various criteria. The association between lower cancer incidence and reduced patient survival can mean that research into some cancers with poor outcomes could be viewed as less "important".[39]

2.29 The LCSA similarly outlined how this funding program disadvantages 'researchers investigating low survival cancers, who generally have less pilot data or proof of concepts than those researching more common cancers with better outcomes'.[40] It submitted that '[t]he NHMRC is not a reliable method for many researchers wishing to secure research funding for low survival cancers to get worthwhile projects off the ground'.[41]

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37 See, for example, The Walter and Eliza Hall Institute of Medical Research (Walter and Eliza Hall Institute), *Submission 126* pp 3–4; Mr Daniel Robinson, *Submission 227*, p. 1.

38 See, for example, Brain Cancer Biobanking Australia, *Submission 119*, p. 2; Ms Marilyn Nelson, *Submission 241*, p. 5; Ms Michelle Stewart, Head of Research Strategy, Cure Brain Cancer Foundation (CBCF), *Committee Hansard*, 6 June 2017, p. 23; Professor Rosalie Viney, Member, Australian Health Economics Society (AHES), *Committee Hansard*, 29 August 2017, p.2.

39 Children's Cancer Research Unit, The Children's Hospital at Westmead (CCRU), *Submission 88*, p. 2.

40 LSCA, *Submission 90*, p. 2.

41 LSCA, *Submission 90*, p. 2.

2.30 Dr Marina Pajic informed the committee of the difficulties with obtaining NHMRC funding based on her experiences:

> In order to get something to the standard that NHMRC requires to really be competitive, that study pretty much needs to be 80 per cent complete. You need to convince these reviewers that this grant is foolproof, that it will work, and that is not really what research should be all about. It is all about figuring out that, actually, maybe something will not work. That in itself may then be an interesting result that you take further and develop new ideas around. I guess philanthropic money is really where those sorts of studies are currently done, and there is just not a lot of that money around. I am talking about pancreatic cancer researchers in general. I am fortunate enough to have the support of the Garvan Research Foundation, so I have been able to get my studies to that level to get NHMRC and Cancer Australia funding on occasion.[42]

2.31 The Australasian Leukaemia and Lymphoma Group (ALLG) noted that, in its experience, the NHMRC model in place prior to 25 May 2017 'favour[ed] those cancers that attract more non-government funding'. The ALLG observed that those cancers which attract non-government funding, have elements of:

- public "popularity" and prominence;
- commerciality i.e. where industry has a vested interest in a commercial pipeline; and
- potential commercialisation of intellectual property.[43]

2.32 However, in its submission, Research Australia suggested another reason why this correlation between non-government and NHMRC funding exists: that is, '[t]he NHMRC typically only funds the direct costs of research, leaving the organisation undertaking the research to meet the indirect research costs from other sources', such as philanthropic funding.[44] An explanation of this reasoning was provided:

> 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.

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42 Dr Marina Pajic, Group Leader, Garvan Institute of Medical Research, *Committee Hansard*, 7 June 2017, p. 53.

43 Australasian Leukaemia and Lymphoma Group (ALLG), *Submission 121*, p. 1.

44 Research Australia, *Submission 122*, p. 7.

> 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.[45]

2.33 In its submission to this committee, the Victorian Comprehensive Cancer Centre (VCCC) also discussed the significance of philanthropic funding:

> Philanthropic sources of funding are divided between patient support services and grants for research and these funds can make a significant impact on preliminary research activity. Higher levels of philanthropic funding for the various charitable cancer foundations has typically been related to (i) higher survival rate cancers, where survivors are active in fundraising to "give back" to the field, and (ii) high incidence cancers, where a large pool of affected individuals and families can be leveraged for philanthropic donations. Low incidence and low survival cancers do not have these resources and moreover, there may be social stigma related to the cancers, e.g. lung and brain cancers.[46]

2.34 Although the VCCC did not consider that there was any 'systemic bias' in the NHMRC model, asserting that '[t]he process of scoring to assess NHMRC applications is rigorous and robust',[47] it was acknowledged that:

> …the success rates of applications reflect the far greater pool of resources available to researchers working in certain areas, e.g. breast cancer, that supports them being successful researchers who will in turn have greater success at NHRMC, i.e. it is the funding of preliminary work, which requires scientists, expendables and infrastructure, that results in a high-scoring funding application. It is also this funding that can enhance track record and demonstrate that a research group can complete the project. This tends to be in the cancer types that have already shown research success and improved outcomes (which are more noteworthy than failures in poor outcome diseases), further compounding the disparity between highly-funded and low-funded research.[48]

2.35 Research Australia therefore proposed that the government should fully fund indirect costs of research on the basis that this:

> …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.[49]

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45 Research Australia, *Submission 122*, p. 7.

46 Victorian Comprehensive Cancer Centre (VCCC), *Submission 114*, p. 2.

47 VCCC, *Submission 114*, p. 1.

48 VCCC, *Submission 114*, pp 1–2.

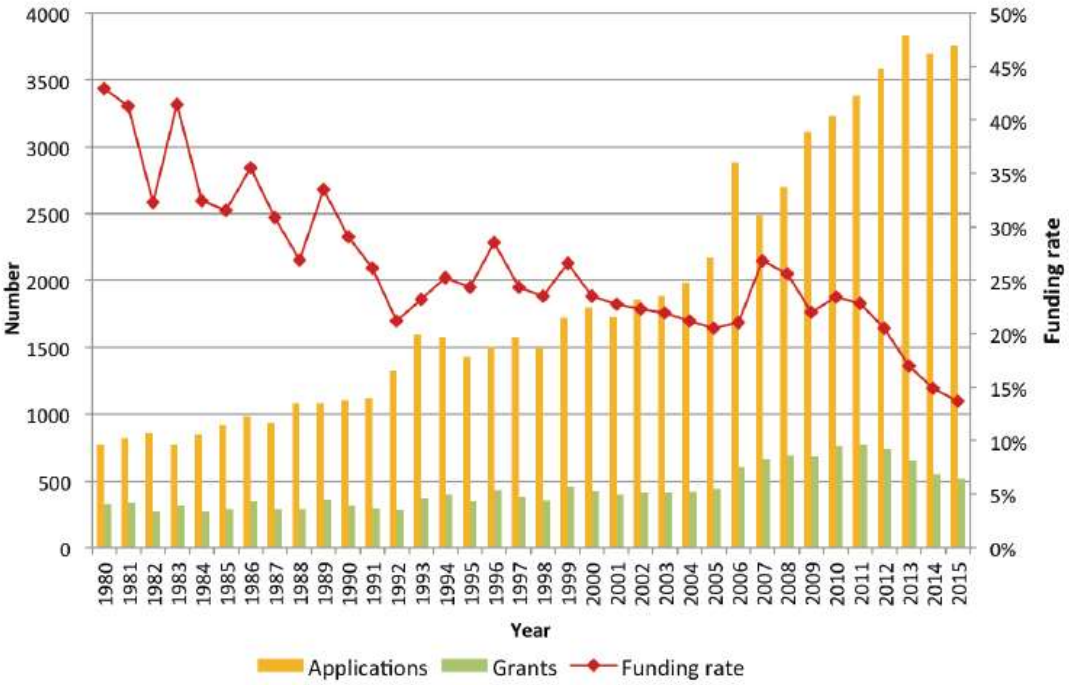
49 Research Australia, *Submission 122*, pp 7–8.

Changes to the NHMRC funding structure

2.36 On 28 January 2016, the NHMRC CEO, Professor Kelso, announced 'an over-arching review of the structure of NHMRC's grant program',[50] which was considered necessary for a number of reasons.

2.37 One reason was the decrease in funding for most of the NHMRC's funding schemes from 2012 to 2015,[51] which created 'a hypercompetitive environment, and [maybe] lead to research proposals targeting low survival rate cancers being increasingly disadvantaged'.[52] This is illustrated by the following example of the Project Grants scheme at Figure 3.

Figure 3: Rising application numbers and falling funding rates in the Project Grants scheme, 1980 – 2015[53]



2.38 Further, there was also 'widespread concern that the high volume of applications for NHMRC funding is having a range of negative effects on Australian health and medical research' including that:

50 NHMRC, *Reviewing the structure of NHMRC's grant program*, 16 May 2016, https://www.nhmrc.gov.au/media/nhmrc_updates/2016/reviewing-structure-nhmrc-s-grant-program (accessed 12 October 2017).

51 NHMRC, *Structural Review of NHMRC's Grant Program: Consultation Paper*, July 2016, p. 10.

52 CCRU, *Submission 88*, p. 1.

53 NHMRC, *Structural Review of NHMRC's Grant Program: Consultation Paper*, July 2016, p. 10.

- Researchers are spending a substantial period each year preparing grant applications that will not be funded, despite many being of sufficient quality to be funded.
- The load on peer reviewers (most of whom are themselves researchers) has become excessive for the number of grants funded.
- Early and mid-career researchers, especially women, may feel discouraged from pursuing a research career.
- Applicants are more likely to propose, and peer reviewers are more likely to favour, "safe" research to the detriment of innovation.
- The low likelihood of funding is driving further increases in application numbers as researchers seek to improve their chances of obtaining a grant, exacerbating the situation.[54]

2.39 The NHMRC's Research Committee, after considering a range of options, reached the conclusion 'that commonly suggested changes to existing funding schemes would not achieve a sufficient reduction in application numbers' that would overcome such issues.[55]

2.40 Indeed, in 2015, many submitters to the NHMRC's public consultation on Current and Emerging Issues for NHMRC Fellowship Schemes called for an overarching review of the NHMRC's grant program.[56]

2.41 The review therefore had the aim of determining:

> …whether the suite of funding schemes can be streamlined and adapted to current circumstances, while continuing to support the best Australian research and researchers for the benefit of human health.[57]

2.42 On 14 July 2016, the NHMRC released a public consultation paper on the review, and public forums were also held in several capital cities.[58]

2.43 During the process of the NHMRC's review into its funding structure, an Expert Advisory Group 'provided advice and assistance to NHMRC in examining the

54 NHMRC, *Structural Review of NHMRC's Grant Program: Consultation Paper*, July 2016, p. 10.

55 NHMRC, *Structural Review of NHMRC's Grant Program: Consultation Paper*, July 2016, p. 11.

56 NHMRC, *Structural Review of NHMRC's Grant Program: Consultation Paper*, July 2016, p. 11.

57 NHMRC, *Structural Review of NHMRC's Grant Program*, 2 June 2017, https://www.nhmrc.gov.au/grants-funding/structural-review-nhmrc-s-grant-program (accessed 12 October 2017).

58 NHMRC, *Structural Review of NHMRC's Grant Program - Public Consultation*, 2 June 2017, https://www.nhmrc.gov.au/grants-funding/structural-review-nhmrc-s-grant-program/structural-review-nhmrc-s-grant-program (accessed 12 October 2017).

current grant program and possible alternative models'.[59] The CEO subsequently drew on its advice in formulating the new funding structure, as well as that of the NHMRC Research Committee, the NHMRC Council, Health Translation Advisory Committee, Health Innovation Advisory Committee and the Principal Committee Indigenous Caucus.[60]

2.44 The NHMRC's restructured funding program, an overview of which appears at Table 3, was announced on 25 May 2017[61] and aims to:

- encourage greater creativity and innovation in research,
- provide opportunities for talented researchers at all career stages to contribute to the improvement of human health, and
- minimise the burden on researchers of application and peer review so that researchers can spend more time producing high quality research.[62]

2.45 In summary:

> The restructured program will comprise Investigator Grants, Synergy Grants, Ideas Grants and Strategic and Leveraging Grants. Limits will also be placed on the number of grants an individual researcher can apply for or hold.
>
> Investigator Grants, Synergy Grants and Ideas Grants will replace Fellowships, Program Grants and Project Grants[63]

59 NHMRC, *Structural Review of NHMRC's Grant Program*, 2 June 2017, https://www.nhmrc.gov.au/grants-funding/structural-review-nhmrc-s-grant-program (accessed 12 October 2017).

60 NHMRC, *Structural Review of NHMRC's Grant Program*, 2 June 2017, https://www.nhmrc.gov.au/grants-funding/structural-review-nhmrc-s-grant-program (accessed 12 October 2017).

61 The Hon. Greg Hunt MP, 'Medical research reforms to improve our future health, *Media Release*, 25 May 2017.

62 NHMRC, *The Changes*, 21 September 2017, https://www.nhmrc.gov.au/restructure/changes (accessed 12 October 2017).

63 NHMRC, *The Changes*, 21 September 2017, https://www.nhmrc.gov.au/restructure/changes (accessed 12 October 2017).

Table 3: Overview of NHMRC's restructured grant program[64]

Grant type	Investigator Grants	Synergy Grants	Ideas Grants	Strategic and Leveraging Grants
Purpose	To support the research programs of outstanding investigators at all career stages	To support outstanding multidisciplinary teams of investigators to work together to answer major questions that cannot be answered by a single investigator.	To support focussed innovative research projects addressing a specific question	To support research that addresses identified national needs
Duration	5 years	5 years	Up to 5 years	Varies with scheme
Number of Chief Investigators	1	4-10	1-10	Dependent on individual scheme
Funding	Research support package (RSP) plus optional salary support	Grant of a set budget ($5 million)	Based on the requested budget for research support	Dependent on individual scheme
Maximum number of applications allowed per round*	1	1	2	Not capped relative to Investigator, Synergy and Ideas Grants. Dependent on individual scheme.
Maximum number of each grant type that can be held**	1	1	Up to 2**	Not capped relative to Investigator, Synergy and Ideas Grants. Dependent on individual scheme.
Indicative MREA allocation	About 40%	About 5%	About 25%	About 30%

* A maximum of two applications per round can be submitted by any individual across the Investigator, Synergy and Ideas Grant schemes. I.e. individuals may only apply for one Investigator Grant and/or one Synergy Grant and/or up to two Ideas Grants in a given application round.

** A maximum of two grants can be held concurrently, by any individual, with the following exceptions and conditions: (1) individuals who hold two Ideas Grants can hold concurrently a Synergy Grant, (2) individuals who hold up to two Ideas Grants can apply for, and hold an Investigator Grant, but their RSP will be discounted until the Ideas Grant/s have ended and (3) individuals may apply for an Investigator Grant concurrently with an Ideas Grant, and if both applications are successful only the Investigator Grant will be awarded.

64 NHMRC, *The Changes*, 21 September 2017, https://www.nhmrc.gov.au/restructure/changes (accessed 12 October 2017).

2.46 In speaking specifically to the Ideas Grants, Professor Kelso informed the committee that this scheme replaces some of what the Projects Grants scheme achieved, 'but in a more effective way'.[65] Professor Kelso continued:

> The purpose of this scheme is to focus on research which is highly innovative, creative and does not require that somebody has a long track record of research, which is an impediment for many people getting started, attempting to change fields or addressing an important new question. Of course, it's still going to be highly competitive, it's going to be highly rigorous but it will have a different flavour from the current Project Grants scheme, which has become increasingly competitive, such that people's track records have become a very important driver in that scheme. So I'm very optimistic that the Ideas Grants scheme is going to fill an important gap in our current range of schemes.[66]

2.47 Dr David Whiteman of the QIMR Berghofer Medical Research Institute welcomed that the Ideas Grants were 'less focussed on track record and more focussed on innovation', and acknowledged that while it is not a large pool of money, 'it is a pool of money to address the issue of innovation and ensure that innovative cutting-edge ideas from younger early-career investigators get picked up'.[67]

2.48 In speaking to the new five year grants for research, Professor Linda Richards considered this a significant improvement compared to the previous three-year funding structure, noting that this:

> …is a huge step forward for everybody in terms of the amount of time writing grants and the amount of time reviewing grants and also the amount of time it takes to do high-quality research. You cannot do this in a three-year funding cycle. It is just too short, especially for an organ system like the brain, because the work is slow and time-consuming and it takes time to do quality research. One thing though is that the NHMRC does have a fourth category, which is for targeted research, and I would implore you that brain research, in particular brain cancer, is one of those areas that we should be targeting in this country.[68]

2.49 Dr Jens Bunt elaborated:

> It is really hard to get long research programs, because most of the project grants are for three years. Sometimes setting up something ambitious or that is more risky takes more time. For instance, even though we did not have funding for it, we invested three years to develop a mouse model. It took us three years to get the exact model to mimic certain cancer development. It is really hard to get funding for those kinds of things and sometimes you

65 Professor Kelso, NHMRC, *Committee Hansard*, 29 August 2017, p. 28.

66 Professor Kelso, NHMRC, *Committee Hansard*, 29 August 2017, p. 28.

67 Dr David Whiteman, Deputy Director, QIMR Berghofer, *Committee Hansard*, 6 June 2017, p. 43.

68 Professor Linda Richards, Deputy Director, Research, Queensland Brain Institute (QBI), The University of Queensland (UQ), *Committee Hansard*, 6 June 2017, p. 14.

> have to think far ahead and invest a lot in developing techniques and novel ideas that do not really directly fit in a project realm. There is always an assumption of a small group of people working on something that is finished within a certain set time. Whereas we, especially with rare cancers, because we do not know that much yet, need to really develop these things with multiple people from multiple different disciplines to work on it. It is really hard to get sufficient scientific funding for that. I think this would also help. But at the moment we have to think in packages of three years, which makes it harder.[69]

2.50 Ms Emma Raymond also informed the committee that Wesley Medical Research had to cease collecting samples, identifying the lack of longevity of funding as a problem:

> The problem is that people give you the money to set something up and give you the infrastructure and the equipment, but there is no longevity, so there is no funding to continue what we are doing. I have seen a lot of biobanks go out of business when they have lost their funding from the NHMRC. The problem is that we have a duty of care to these patients. We have collected their samples to help other patients. If we lose our funding, then we have to basically shut the doors, which is what happened at [the University of Queensland] with their brain bank.[70]

2.51 Research Australia, which postulated that the changes to the NHMRC funding structure 'are positive for the subject of this inquiry',[71] also spoke to the importance of secure long term funding for research. Research Australia stated that in order to see the greatest outcomes, research must be funded for an extended period of time, as '[r]esearch, by its nature, is a long term prospect', and provided the following example:

> …to develop a new drug, from the initial stages through to the end, takes anywhere between 10 and 15 years and can cost up around $3 billion. So these are very intensive processes that need support over a long period.[72]

2.52 Although the overall changes to the grant program have been welcomed by some, Dr Elizabeth Johnson of the VCCC warned that the NHMRC's 'capacity to support multidisciplinary research may have been reduced' by these changes, explaining that:

> The focus is shifting away a little bit from the old fashioned program grants, where you got a number of multidisciplinary teams, a number of different people who had come from different institutions, who worked together to support a particular research initiative. They typically tended to be a bit bigger. We have yet to see how the restructure plays out, but the NHMRC funding structure might not now be the ideal support for the type

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69 Dr Jens Bunt, Research Fellow and Team Leader, NFI Research Lines, Brain Development and Disorders Laboratory, QBI, UQ, *Committee Hansard*, 6 June 2017, p. 15.

70 Ms Emma Raymond, Theme Leader, Cancer, Wesley Medical Research, *Committee Hansard*, 6 June 2017, p. 30.

71 Mr Mullins, Research Australia, *Committee Hansard*, 7 June 2017, p. 43.

72 Mr Mullins, Research Australia, *Committee Hansard*, 7 June 2017, p. 46.

> of multidisciplinary approach that we need to really tackle [survival rates] properly.[73]

Cancer Australia

2.53 Cancer Australia, a statutory body established in 2006 pursuant to the *Cancer Australia Act 2006*, is 'the lead national cancer control agency' and 'aims to reduce the impact of cancer, address disparities and improve outcomes for people affected by cancer by leading and coordinating national, evidence-based interventions across the continuum of care'.[74]

2.54 Cancer Australia has the following functions:

> (a) to provide national leadership in cancer control;
>
> (b) to guide scientific improvements to cancer prevention, treatment and care;
>
> (c) to coordinate and liaise between the wide range of groups and health care providers with an interest in cancer;
>
> (d) to make recommendations to the Commonwealth Government about cancer policy and priorities;
>
> (e) to oversee a dedicated budget for research into cancer;
>
> (f) to assist with the implementation of Commonwealth Government policies and programs in cancer control;
>
> (g) to provide financial assistance, out of money appropriated by the Parliament, for research mentioned in paragraph (e) and for the implementation of policies and programs mentioned in paragraph (f);
>
> (h) any functions that the Minister, by writing, directs Cancer Australia to perform.[75]

2.55 In its submission, Cancer Australia noted that it performs its function to oversee a dedicated budget for research into cancer[76] through administration of the Priority-driven Collaborative Cancer Research Scheme (PdCCRS).

2.56 The PdCCRS, established in 2007, 'brings together government and other funders of cancer research to coordinate, co-fund and maximise the number of cancer research grants funded in Australia',[77] and was established:

> …in order to:
>
> - better coordinate funding of priority-driven cancer research;

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73 Dr Elizabeth Johnson, Program Manager, VCCC, *Committee Hansard*, 7 June 2017, p. 41.

74 Cancer Australia, *About us*, https://canceraustralia.gov.au/about-us (accessed 16 October 2017).

75 *Cancer Australia Act 2006*, ss. 7(1).

76 *Cancer Australia Act 2006*, para. 7(1)(e).

77 Cancer Australia, *Submission 129*, p. 3.

- foster collaborative cancer research and build Australia's cancer research capacity, and
- foster consumer participation in cancer research, from design to implementation.[78]

2.57 In determining which research programs to fund, Cancer Australia uses 'an evidence based approach' to fill gaps in funding, which was described to the committee by Dr Paul Jackson:

> We look at the national pattern of funding to cancer research, which includes the funding that is provided from both national and international sources, and, using that profile, we examine the funding that goes to different tumour types as well as the funding across the broad areas of the research spectrum—the main areas of the funding to where that project goes. We then use that evidence to identify opportunities for us to make strategic investments where there are gaps or opportunities to further research. That, for example, can be in tumours which may be of high burden and poor survival, where there are opportunities to strategically invest to address that.[79]

2.58 Dr Jackson informed the committee that in determining which applications to fund, a merits-based approach is used, such that Cancer Australia funds:

> …from the top-ranked merit based application downwards. We maximise the amount of funding, or the number of grants that we're able to fund, through collaborative funding with our funding partners in the scheme. We start from the top down. Once the funding has ended, that's where we have to stop funding.[80]

2.59 Dr Whiteman commended Cancer Australia on this approach:

> I think the activities that Cancer Australia has done in just looking back and saying: 'What have we funded previously? Does that reflect where we want to invest our funding?' are very helpful, because they then put the spotlight on neglected areas of research, including low-survival cancers. I think there is a mood for recognising where there are deficits in funding, and then looking for mechanisms to correct that.[81]

2.60 Other witnesses described the type of funding they receive from Cancer Australia, and the positive impact this has had on their research.[82] For example, Ms Delaine Smith of the ALLG informed the committee that:

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78 Cancer Australia, *Submission 129*, p. 3.

79 Dr Paul Jackson, Acting General Manager, Knowledge Management, Cancer Australia, *Committee Hansard*, 29 August 2017, p. 21.

80 Dr Jackson, Cancer Australia, *Committee Hansard*, 29 August 2017, p. 21.

81 Dr Whiteman, QIMR Berghofer, *Committee Hansard*, 6 June 2017, p. 39.

82 See, for example, Mrs Tricia Berman, Secretary, Brain Tumour Alliance Australia (BTAA), *Committee Hansard*, p. 47.

> …the ALLG, and now 13 other cancer trial groups around Australia, have been able to have funding come straight from Cancer Australia. That is about half a million dollars a year. The infrastructure that it supports is very specific because Cancer Australia is very specific about how it can be spent. So it goes towards the activities that develop clinical trials. For us, in the ALLG, we utilise that funding on EFT and on roles and positions that help prepare the clinical trial protocol. The protocol is the instruction document that is going to go to the hospital to tell them what to do in a very methodical and meticulous way. You cannot understate the importance of preparation. Preparation is key.[83]

2.61 However, the committee also heard that Cancer Australia could have a lead role with respect to 'developing, implementing and maintaining' a sustained focus on LSR cancers.[84] Further discussion about a national strategy for LSR cancers appears at chapter 5.

The Medical Research Future Fund

2.62 The MRFF, which operates pursuant to the *Medical Research Future Fund Act 2015* (MRFF Act), was established as part of the 2014–15 Federal Budget with the purpose of providing:

> …a sustainable source of funding for vital medical research over the medium to longer term. Through the MRFF, the Government will deliver a major additional injection of funds into the health and medical research sector.[85]

2.63 The $20 billion fund 'offers the opportunity to strategically fund research and address national priorities in a cohesive and coordinated way'.[86] The MRFF 'complements existing medical research and innovation funding', such as the NHMRC, the Commonwealth Science Council and the National Innovation and Science Agenda, 'to improve health outcomes by distributing new funding in more diverse ways to support stronger partnerships between researchers, healthcare professionals, governments and the community'.[87]

2.64 The operation of the MRFF is summarised in the MRFF Act as follows:

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83 Ms Delaine Smith, CEO, ALLG, *Committee Hansard*, 7 June 2017, p. 33.

84 Mr James Armstrong, Member, Consumer Advisory Panel, GI-Cancer Institute, Australasian Gastro-Intestinal Trials Group, *Committee Hansard*, 18 May 2017, p. 49.

85 The Department of Health (DoH), *Further information on the Medical Research Future Fund*, 9 May 2017, http://www.health.gov.au/internet/main/publishing.nsf/Content/mrff-more (accessed 11 October 2017).

86 DoH, *Further information on the Medical Research Future Fund*, 9 May 2017, http://www.health.gov.au/internet/main/publishing.nsf/Content/mrff-more (accessed 11 October 2017).

87 DoH, *Further information on the Medical Research Future Fund*, 9 May 2017, http://www.health.gov.au/internet/main/publishing.nsf/Content/mrff-more (accessed 11 October 2017).

> The Medical Research Future Fund consists of the Medical Research Future Fund Special Account and the investments of the Medical Research Future Fund. Initially, the Fund's investments are a portion of the investments of the Health and Hospitals Fund which was established under the *Nation−building Funds Act 2008*. Additional amounts may also be credited to the Medical Research Future Fund Special Account.
>
> The Medical Research Future Fund Special Account can be debited for 3 main purposes:
>
> (a) channelling grants to the COAG Reform Fund to make grants of financial assistance to States and Territories; and
>
> (b) channelling grants to the MRFF Health Special Account to make grants of financial assistance to certain bodies; and
>
> (c) making grants of financial assistance directly to corporate Commonwealth entities.
>
> The Australian Medical Research Advisory Board is established to determine the Australian Medical Research and Innovation Strategy and the Australian Medical Research and Innovation Priorities. The Health Minister takes the Priorities into account in making decisions about the financial assistance that is provided from the Medical Research Future Fund Special Account.
>
> There is a limit on the amount that can be debited from the Medical Research Future Fund Special Account each financial year. The limit, which is called the maximum annual distribution, is determined by the Future Fund Board for each financial year.
>
> The Medical Research Future Fund is invested by the Future Fund Board in accordance with an Investment Mandate given by the responsible Ministers.[88]

2.65 Professor Ian Frazer, Chair of the Australian Medical Research Advisory Board (AMRAB) which determines the Australian Medical Research and Innovation Strategy and the Australian Medical Research and Innovation Priorities pursuant to the MRFF Act,[89] outlined for the committee the differences between the NHMRC and the MRFF:

> The National Health and Medical Research Council largely gives funding out in reply to specific proposals from individual researchers. It does have some priority areas which it uses, but the vast majority of funding is in response to a particular proposal on a particular bit of research determined by the investigator themselves. The Australian Medical Research Advisory Board advisory to the Medical Research Future Fund rather takes the view of top-down driven research where we have recommended to the minister priorities where we believe that research money should be best spent. Therefore, while there might be a call for proposals in due course, at the

88 *Medical Research Future Fund Act 2015*, s. 4.

89 *Medical Research Future Fund Act 2015*, s. 32D–s. 32EA.

> moment the money is being dispersed on the basis of the priorities and strategies that we set when we completed our consultation with the medical research community, the general public and other interested parties in the course of 2016.[90]

2.66 Professor Frazer considered that the MRFF Act provides sufficient flexibility in the granting of funding, specifically in relation to collaboration across institutions:

> Certainly, the funding will have to be administered by one individual organisation which is responsible for its acquittal back to government. But the concept of collaboration in research is pretty much international, of course. Certainly, there is nothing intended about the way that we made the strategy of priorities to suggest that we did not wish to see collaboration. In fact, we positively expected that there would be collaboration and pointed out that the value of collaboration, for example, between different research institutes in this country and overseas, and research institutes and industry, should be positively encouraged.[91]

The 2016–2021 strategy

2.67 Following consultation with the sector and the broader community, and pursuant to the MRFF Act,[92] the AMRAB developed six strategic platforms to underpin the *Australian Medical Research and Innovation Strategy 2016–2021* (the Strategy) that 'capture and group together themes and provide a framework for the [*Australian Medical Research and Innovation Priorities 2016–2018*] to improve research capacity and capabilities in the research sector'.[93] A list of priorities falls under each of these strategic platforms.[94]

2.68 The Strategy also sets out how the MRFF aligns with and compliments the NHMRC, the National Science and Innovation Agenda, and other interests, such as state and territory governments and the private and not-for-profit sectors;[95] as well as the challenges facing the health and medical research sector.[96]

2.69 The strategic platforms of the Strategy are:

- **strategic and international horizons**: funding to support Australian participation and leadership in 'international research projects focusing on

90 Professor Ian Frazer, Chair, Australian Medical Research Advisory Board (AMRAB), *Committee Hansard*, 8 June 2017, p. 48.

91 Professor Frazer, AMRAB, *Committee Hansard*, 8 June 2017, p. 48.

92 *Medical Research Future Fund Act 2015*, s. 32EA.

93 DoH, *Australian Medical Research and Innovation Strategy 2016–2021*, 9 November 2016, p. 7 (tabled 29 August 2017).

94 DoH, *Australian Medical Research and Innovation Strategy 2016–2021*, 9 November 2016, p. 7 (tabled 29 August 2017).

95 DoH, *Australian Medical Research and Innovation Strategy 2016–2021*, 9 November 2016, pp 3–5 (tabled 29 August 2017).

96 DoH, *Australian Medical Research and Innovation Strategy 2016–2021*, 9 November 2016, pp 5–7 (tabled 29 August 2017).

major global health challenges and threats…complimentary to the international collaborative activities of the NHMRC';[97]

- **data and infrastructure**: funding for research that 'enables the planning and implementation' of 'an integrated national health data framework that supports healthcare delivery, service improvement and best practice adoption';[98]
- **health services and systems**: in contrast to the current product and drug focussed medical research and the domination of the acute care experience for research on health interventions, the intention is to bolster 'Australia's capacity in health services and systems research' by, for example, 'investment activities…with the Medicare Benefits Schedule Review Taskforce and new policy and program agendas, such as the Australian Government's Health Care Homes trial';[99]
- **capacity and collaboration**: the focus is research collaboration, to be achieved by 'investing in multi-disciplinary, institute and sector teams', which could extend to collaborative funding, 'by leveraging co-investment from other governments, private and philanthropic interests';[100]
- **trials and translation**: the facilitation of 'non-commercial clinical trials of potential significance', including by supporting NHMRC-accredited Advanced Health Research and Translation Centres;[101] and
- **commercialisation**: supporting 'the creation and brokering of linkages between researchers and industry that are transdisciplinary in nature', noting the need for '[a] two-way exchange of knowledge and expertise in research, and its translation into clinical practice' and better encouragement 'adoption of the requirements for successful commercialisation in both the academic and business environment'.[102]

2.70 Professor Frazer commented that, for the next round of consultations, improvements could be made to AMRAB's processes:

> …we may actually have to get focus groups together and specifically engage, through the recruitment of individuals who would not otherwise

97 DoH, *Australian Medical Research and Innovation Strategy 2016–2021*, 9 November 2016, pp 7–8 (tabled 29 August 2017).

98 DoH, *Australian Medical Research and Innovation Strategy 2016–2021*, 9 November 2016, p. 8 (tabled 29 August 2017).

99 DoH, *Australian Medical Research and Innovation Strategy 2016–2021*, 9 November 2016, p. 8 (tabled 29 August 2017).

100 DoH, *Australian Medical Research and Innovation Strategy 2016–2021*, 9 November 2016, p. 9 (tabled 29 August 2017).

101 DoH, *Australian Medical Research and Innovation Priorities 2016–2018*, 9 November 2016, p. 9 (tabled 29 August 2017).

102 DoH, *Australian Medical Research and Innovation Strategy 2016–2021*, 9 November 2016, p. 10 (tabled 29 August 2017).

> necessarily come forward, to get a more general representation of what the public is interested in. One of the practical realities, of course, is that people become most interested in the health system when they actually need to use it, and yet the vast majority of people out there who might, in the future, benefit from it, do not actually use it at the moment.[103]

2.71 Indeed, Professor Rosalie Viney of the Australian Health Economics Society advocated for an additional injection of funds from the MRFF into health research 'across the board':

> It shouldn't just be in the discovery science; it needs to be across the whole of translation. But I think it's absolutely critical that that is done in a way that maintains the standards of excellence in research, maintains the standards of scientific quality, makes sure that we apply the same well-established principles that organisations like NHMRC have had for peer review and for quality, and that that continues.[104]

2.72 However, Dr Richard De Abreu Lourenco warned that if the MRFF were to be used for discovery research, it could be viewed 'as an implication of support for commercialisation' from the government.[105]

First disbursements

2.73 The first disbursements of the MRFF, implemented in 2016–17, invested $65.9 million:

- **$20 million** for preventive health and research translation projects.
- **$33 million** for clinical trials that will build on Australia's world class research strengths and ensure Australia is a preferred destination for research.
- **$12.9 million** for breakthrough research investments that drive cutting edge science and accelerate research into better and new treatments and cures.[106]

2.74 Professor Terrance Johns of the Brain Cancer Discovery Collaborative, who stated that his institution 'is not a large institution with political clout', noted that '[t]here was no call for grants for MRFF funding' for its first disbursements, and observed that the funds are 'pretty much locked up by the G8 universities'.[107] Professor Johns opined that, at present, the MRFF 'is about political clout'.[108]

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103 Professor Frazer, AMRAB, *Committee Hansard*, 8 June 2017, p. 49.

104 Professor Viney, AHES, *Committee Hansard*, 29 August 2017, p. 6.

105 Dr Richard De Abreu Lourenco, Member, AHES, *Committee Hansard*, 29 August 2017, p. 7.

106 DoH, *Medical Research Future Fund – Overview of the Medical Research Future Fund*, 5 August 2017, http://www.health.gov.au/internet/main/publishing.nsf/Content/medical-research-future-fund-mrff-overview-budget-2017 (accessed 11 October 2017).

107 Professor Terrance Johns, Director, Brain Cancer Discovery Collaborative, *Committee Hansard*, 18 May 2017, p. 25.

108 Professor Johns, Brain Cancer Discovery Collaborative, *Committee Hansard*, 18 May 2017, p. 26.

2.75 However, Mr Peter Orchard, whose organisation CanTeen Australia was a recipient of some MRFF funding, suggested that '[t]o some extent, the MRFF is in its absolute infancy, and so being able to comment on it feels difficult at this stage, other than to say I am very grateful for it'.[109]

2.76 Indeed, Mr Mullins of Research Australia spoke to the benefits of the MRFF:

> …the MRFF funding, with its emphasis on translation, offers new opportunity for advances that will benefit patients. The MRFF, importantly, also has a top-down approach to funding. It is driven by a five-year strategy and priorities, and the latter must explicitly take into account the burden of disease, how to deliver practical benefits to the Australian community and value for money. This must be combined with a focus on funding excellent research, obviously, if it is to be successful, but it provides greater scope for strategically directing funding to particular areas.[110]

Philanthropic funding

2.77 As indicated at paragraphs 2.32–2.33 above, philanthropic funding can be vital to advances for research into LSR cancers, especially when researchers find it difficult to obtain government funding.

2.78 Indeed, it was noted by the ANZCHOG National Patient and Carer Advisory Group that 'oncology units are often largely dependent upon philanthropic and charitable donations' to meet costs associated with enrolment in and compliance with international trials, emphasising that '[c]urrently paediatric centres rely heavily on philanthropy, charities and individual hospital budgets to fund most cancer clinical trials'.[111]

2.79 To illustrate what such funding can achieve, the Mark Hughes Foundation (MHF) outlined that in three years, it has contributed to the following improvements in respect of brain cancer:

- A Brain Cancer Biobank at [the Hunter Medical Research Institute]
- Over $300,000 in project grant funding and various Travel Grants to allow brain cancer researchers attend international conferences to present their work and establish important research collaborations
- A clinical research fellowship in Brain Cancer
- A dedicated Brain Cancer Care Nurse at John Hunter Hospital
- Communal brain cancer research register with Brain Cancer Biobanking Australia[112]

2.80 Further, Professor Mark Rosenthal of the VCCC spoke to the work of the Cure Brain Cancer Foundation (CBCF), a philanthropic organisation focused

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109 Mr Peter Orchard, CEO, CanTeen Australia, *Committee Hansard*, 19 May 2017, p. 6.

110 Mr Mullins, Research Australia, *Committee Hansard*, 7 June 2017, p. 43.

111 ANZCHOG National Patient and Carer Advisory Group, *Submission 125*, p. 7.

112 Mark Hughes Foundation (MHF), *Submission 113*, p. 3.

exclusively on brain cancer, in providing financial assistance for brain cancer research:

> The [CBCF] has done terrifically well through, really, one individual driving that over many years, but they now have a very established philanthropic organisation that runs professionally and relatively independently. We have made sure that there is rigour to their grant application process and the grants that have been given out. It is not in competition with NHMRC. It has grown because of the need for it. It would be great if we did not have to have philanthropic funding, but actually we are lucky in brain that at least there is some. We have only had one round of grants, which total up to $2 million, I think.[113]

2.81 However, Associate Professor Gavin Wright identified a significant issue with attracting philanthropic funding for LSR cancers, namely, the lack of survivors:

> The trouble with the philanthropic side of things is often you need survivors, who generate a lot of push for these sorts of things. They go to companies. The catch 22 is that, if you have a poor-survival cancer, you do not have many survivors. If it is affecting a lower socioeconomic group, you do not have the movers and shakers.[114]

2.82 Furthermore, as Dr Johnson noted, 'success breeds success' in terms of the growth of philanthropic cancer support groups, observing that:

> Once you have a critical mass of funding you can then do more with it—you can advertise more and you can grow your foundations more. There are numerous lesser-known small cancer foundations which really do exist on the smell of an oily rag.[115]

2.83 The committee therefore heard calls for various improvements in respect of philanthropic funding. For example, in addition to the recommendation by Research Australia at paragraph 2.35 above that the government fund indirect costs of research in order to 'allow more philanthropic funding to be directed to support novel early stage research and early career researchers',[116] Professor Guy Eslick called for greater philanthropy from 'wealthy Australian businesses and individuals'.[117]

2.84 In his submission, Professor Eslick drew a contrast between the philanthropic funding Harvard University received for research during his post-doctoral training at Harvard ($100 million), compared to that received by the University of Sydney in that same week ($10 million).[118] Professor Eslick suggested that the government could

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113 Professor Mark Rosenthal, Clinical Trials Lead, VCCC, *Committee Hansard*, 7 June 2017, p. 39.

114 Associate Professor Gavin Wright, Research and Education Lead, Lung Cancer, VCCC, *Committee Hansard*, 7 June 2017, p. 39.

115 Dr Johnson, VCCC, *Committee Hansard*, 7 June 2017, p. 40.

116 Research Australia, *Submission 122*, p. 8.

117 Professor Guy Eslick, *Submission 51*, p. 9.

118 Professor Eslick, *Submission 51*, pp 9–10.

encourage philanthropists to donate to universities and research institutions by offering greater incentives.[119]

2.85 The committee also received the following suggestions for improvement with respect of philanthropic funding:

- the Lung Foundation Australia called for the '[p]hilanthropic community to establish specific targets for donations to lung cancer research';[120]
- the MHF called for '[t]argeted Federal and state funding towards brain tumour research, leveraged with funds from philanthropic agencies' to enhance productivity in the field of brain cancer research;[121] and
- Ovarian Cancer Australia recommended the development of 'a national strategy for coordinating the planning and funding of cancer research across the government, medical, health, research and philanthropic communities'.[122]

2.86 Despite the evidence from a number of submitters about their difficulty in securing philanthropic funding, Mr Todd Harper of the Cancer Council Victoria informed the committee that his organisation had not found it difficult to get philanthropic support for research into LSR cancers, asserting that:

> …we have found that there is both an appetite amongst philanthropy to invest in the haematology of less common cancers and in the high-risk, high-return research. I think what is critical here though is that one of the things that makes it more likely that philanthropy would fund these is if they can have assurances over the quality or the rigour of the scientific processes that assess those proposals. I think there is opportunity to bring together the best scientific minds to assess high-quality proposals that can be funded by philanthropic organisations like ours, or indeed others. I think government can also play a role in providing seeding or cooperative funding to enhance the chances of those programs being successful and the chances of those programs being successfully funded.[123]

2.87 However, the committee also heard that '[p]hilanthropy will only go so far': in speaking of the establishment of a centre for research excellence, although the Walter and Eliza Hall Institute of Medical Research had benefitted from philanthropic funding when NHMRC funding was not available, Professor Clare Scott noted that '[g]overnment funding would allow us to entrench these approaches in Australian medicine'. [124]

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119 Professor Eslick, *Submission 51*, p. 10.

120 Lung Foundation Australia, *Submission 89*, Annexure: *Improving outcomes for Australians with lung cancer. A Call to Action*, p. 4.

121 MHF, *Submission 113*, p. 4.

122 Ovarian Cancer Australia, *Submission 242*, p. 4.

123 Mr Todd Harper, CEO, Cancer Council Victoria, *Committee Hansard*, 18 May 2017, p. 31.

124 Professor Clare Scott, Head, Rare Cancer Research, Walter and Eliza Hall Institute, *Committee Hansard*, 4 August 2017, p. 13.

Pharmaceutical funding

2.88 A number of witnesses, whose clinical trial research was funded by pharmaceutical companies, outlined for the committee the importance of funding from pharmaceutical companies for cancer research.[125] However, as the below evidence demonstrates, many witnesses were also critical of the reluctance of pharmaceutical companies to become involved in drug development for people with LSR cancers.

2.89 Roche Products Pty Limited (Roche), a research-based healthcare company focussing on pharmaceuticals and diagnostics, discussed the role of pharmaceutical companies in improving survival rates for LSR cancers:

> The pharmaceutical industry is a critical component of the innovation ecosystem. Not only does industry contribute to basic research and takes the lead in taking medicines through regulatory and reimbursement processes, it is also the leading funder of clinical trials.[126]

2.90 Roche identified that improving survival outcomes for people with LSR cancers is dependent on a number of factors including overcoming barriers to participation in clinical trials (by clinicians as well as patients), and affordable access to treatments through the PBS.[127] Roche identified that '[b]reakthroughs in personalised medicine and immunotherapy are offering hope to patients with both common and rare cancers – yet these products face many challenges in navigating the reimbursement system'.[128]

2.91 Indeed, a recent Deloitte Access Economics (Deloitte) report noted that currently, 'only a small proportion of the potential indications for which immunotherapies are able to be used in cancer treatment receive subsidised funding from the Government', and as these therapies are expensive to develop and produce, treatments 'are prohibitively expensive for many patients who seek to self-fund'.[129] A further discussion of this report, and its recommendations, appears at chapter 5.

2.92 Medicines Australia—'the Australian peak body for the discovery-driven pharmaceutical industry'—identified other challenges for pharmaceutical companies particularly in respect of the policy and access environment:

> The broader policy environment is also challenging the investment decisions made by pharmaceutical companies. Increasing levels of uncertainty caused by a single payer system, as well as inconsistent approaches to intellectual property, aggressive pricing policies and an

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125 See, for example, Mr Peter Kempen, Chairman of the Board, ALLG, *Committee Hansard*, 7 June 2017, p. 35; Professor David Thomas, Director, The Kinghorn Cancer Centre; Head, Cancer Research Division, Garvan Institute (Garvan Institute), *Committee Hansard*, 8 June 2017, p. 32.

126 Roche Products Pty Limited (Roche), *Submission 124*, p. 6.

127 Roche, *Submission 124*, p. 3.

128 Roche, *Submission 124*, p. 3.

129 Deloitte Access Economics (Deloitte), *The New Wave of Immunotherapy Cancer Medicines – The Untapped Potential for Australians*, October 2017, p. 61.

> unpredictable policy environment, are among the issues which Medicines Australia finds to be of some concern.[130]

2.93 The committee also received evidence that there is a limited incentive for pharmaceutical companies to fund clinical trials for LSR cancers,[131] with one witness describing the lack of funding for brain tumour research 'very disappointing'.[132] Other barriers to clinical trials distinct from pharmaceutical funding that are faced by people with LSR cancers is examined in chapter 3.

2.94 Speaking to the involvement of pharmaceutical companies in drug development, Professor Richards asserted that 'it is unethical not to think about those patients [with LSR cancers] and not to be trying to develop treatments for them', arguing that '[t]hat is where government has to step in'.[133] Professor Richards stated that:

> …pharmaceutical companies have been turning away from drug development for brain, partly because we, firstly, did not know enough about the pathways involved to make the clinical trials effective. Also, for rare diseases, of course, the market is not there for the company to want to invest in a drug that is going to be used by a small number of patients.[134]

2.95 The ANZCHOG National Patient and Carer Advisory Group also recognised the importance of return on investment for pharmaceutical companies, submitting that '[t]here is little economic incentive for pharmaceutical companies to fund paediatric cancer trials' as childhood cancers are 'made up of rare and ultra-rare diseases'.[135]

2.96 This was also reflected by Mrs Therese Townsend, a pathology scientist who has a neuro-endocrine tumour:

> The costs of running such trials are disproportionate to the potential profit when there are few potential "customers". When those who may benefit have inherently poor prognoses, courses of treatment are likely to be short, and this further minimises the return on research investment. Hence there is no financial incentive for private enterprise to conduct such trials, especially in Australia due to its decentralisation and small population base.[136]

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130 Medicines Australia, *Submission 141*, p. 9.

131 See, for example, CanTeen Australia, *Submission 128*, p. 3; Dr Robert De Rose, Co-founder, The Isabella and Marcus Paediatric Brainstem Tumour Fund (The Isabella and Marcus Fund), *Committee Hansard*, 7 June 2017, p. 59.

132 Professor Walker, *Committee Hansard*, 6 June 2017, p. 48.

133 Professor Richards, QBI, UQ, *Committee Hansard*, 6 June 2017, p. 20.

134 Professor Richards, QBI, UQ, *Committee Hansard*, 6 June 2017, p. 20.

135 ANZCHOG National Patient and Carer Advisory Group, *Submission 125*, p. 7.

136 Mrs Therese Townsend, *Submission 46*, p. 1.

2.97 Dr Chris Fraser spoke to two barriers to participating in international clinical trials: first is the cost of participation, and second, the increasing requirement to partner with pharmaceutical companies.[137] Dr Fraser elaborated on this second barrier:

> Historically, this was very much an academic pursuit and there were not new drugs, as I outlined, so we were able to do this amongst ourselves. As these new drugs are developed, we increasingly have to partner with pharma companies. Australia is not a big market. It is expensive for them to open these trials in Australia. There may be only one, two or three Australian patients that are eligible for a particular trial. So we need to work out a structure that means we can still participate in these trials. The first step to that is to make sure that we have a very robust clinical trials infrastructure so that we are up and ready to start these trials so the pharmaceutical companies know that the infrastructure and the organisations are there to make sure that the process will run smoothly.[138]

2.98 Indeed, the Garvan Institute of Medical Research/The Kinghorn Cancer Centre/The Garvan Research Foundation (Garvan Institute) identified that '[t]he cost of drug development, which must be recouped by the pharmaceutical industry, already limits access of some patients to important treatment options' and outlined the significant cost of running trials:

> The financial costs of conducting clinical trials have doubled every nine years for the past 50 years. The estimated combined costs per patient in a cancer clinical trial rose from less than US$10,000 to around US$47,000 between 1980 and 2011. The average phase 2 study of 40 patients costs upwards of US$2-10M, while the average phase 3 study costs upwards of US$40M. Average development costs are estimated at around US$3.6 billion dollars per drug.[139]

2.99 However, the Garvan Institute also informed the committee about the alternative ways it has engaged with pharmaceutical companies to conduct clinical trials. In order to minimise the barriers to engagement with pharmaceutical partners in respect of its Molecular Screening and Therapeutics (MoST) study, the Garvan Institute sought only:

> …access to study drugs for each module and for engagement with the pharmaceutical partner in data interpretation, as well as decision-making regarding expansion of a drug–disease cohort in which a significant signal of activity has been identified.[140]

2.100 Professor David Thomas of the Garvan Institute explained how this system works in practice:

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137 Dr Chris Fraser, Chair, Australian and New Zealand Children's Haematology and Oncology Group (ANZCHOG), *Committee Hansard*, 7 June 2017, p. 20.

138 Dr Fraser, ANZCHOG, *Committee Hansard*, 7 June 2017, p. 20.

139 Garvan Institute, *Submission 34*, p. 9 (citations omitted).

140 Garvan Institute, *Submission 34*, p. 9.

> …we invest in drugs by where they are arise. If you invest in breast cancer, you authorise and reimburse drugs on the basis that it works in breast cancer, and that drives the way in which pharma invest. The problem is that many of these drugs work across a whole range of cancers, because a whole range of cancers have this particular common molecular abnormality. A molecular taxonomy is required. That requires molecular screening. Pharma cannot invest in screening 10,000 people to find 20 to treat, but we can. If we can match our research investment with the opportunities from pharma, so we can create a healthy model of collaboration with the benefit of pharma in mind but also getting patients onto trials, that is a virtuous cycle.[141]

2.101 Further discussion about clinical trials appears at chapter 3, and further discussion about the treatment of cancer through personalised medicine and immunotherapies is found in chapter 5.

The TGA, PBAC and PBS

2.102 In order to understand the challenges that face people with LSR cancers, and why those 30 per cent of cancer deaths in Australia that are 'a consequence of the lack of investment in research' receive six per cent of all drug funding,[142] it is necessary to briefly examine the key mechanisms that determine affordable access to medicines.

2.103 Medicines Australia stated that '[r]are disease molecules are often not well-accommodated by the current processes',[143] and opined that 'improved access to medicines via the PBS is the best way forward'.[144] Medicines Australia further suggested that:

> As the national therapeutic goods regulatory reform agenda has resulted in welcome amendments to the definition of such things as ‘orphan’ drugs, and will speed up regulatory approvals in certain cases of high unmet need, it is now also time to review the reimbursement processes for those medicines.[145]

2.104 However, Professor Andrew Wilson, Chair of the PBAC, informed the committee that an 'orphan drug' is not a PBAC designation, but one made by the TGA, and further noted that 'basically it's a situation where you've got a disease where there

141 Professor Thomas, Garvan Institute, *Committee Hansard*, 8 June 2017, p. 33.

142 Professor Thomas, Garvan Institute, *Committee Hansard*, 8 June 201, p. 31.

143 Medicines Australia, answers to questions on notice, 8 June 2017, (received 20 October 2017), p. 1.

144 Medicines Australia, answers to questions on notice, 8 June 2017, (received 20 October 2017), p. 2.

145 Medicines Australia, answers to questions on notice, 8 June 2017, (received 20 October 2017), p. 1.

aren't very many other treatments available for it—a rare disease without any other treatments for it—although sometimes it's also used where there are no other drugs'.[146]

2.105 Figure 4 sets out how the Health Technology Assessment (HTA) process—performed by the TGA, Medical Services Advisory Committee (MSAC), PBAC and the Prostheses Advisory Committee, which provide advice to the Australian government—works in practice.

2.106 As can be seen, the first step in the HTA process is for a medicine to receive regulatory approval from the TGA. This will be required for the use of a medicine by a patient unless: a medical practitioner has been granted authority to dispense a drug to specific patients with a medical condition; a patient has been approved for access to a drug, which is determined on a case by case basis; or there are specific circumstances to warrant access to the drug.[147]

2.107 Once a drug has been approved by the TGA, a sponsor may submit an application to the PBAC, which then determines whether a medicine will be listed on the PBS.[148] As Professor Wilson informed the committee, the PBAC, established pursuant to the *National Health Act 1953*[149] 'to consider the effectiveness and the cost of the proposed medicine compared with existing alternative therapies':[150]

> …cannot make a positive recommendation for a medicine that is substantially more costly than an alternative medicine unless we're satisfied the proposed medicine also provides a significant improvement in health for at least some population.[151]

146 Professor Andrew Wilson, Chair, Pharmaceutical Benefits Advisory Committee (PBAC), *Committee Hansard*, 29 August 2017, p. 18.

147 Senate Community Affairs References Committee, *Availability of new, innovative and specialist cancer drugs in Australia*, September 2015, pp 10–11.

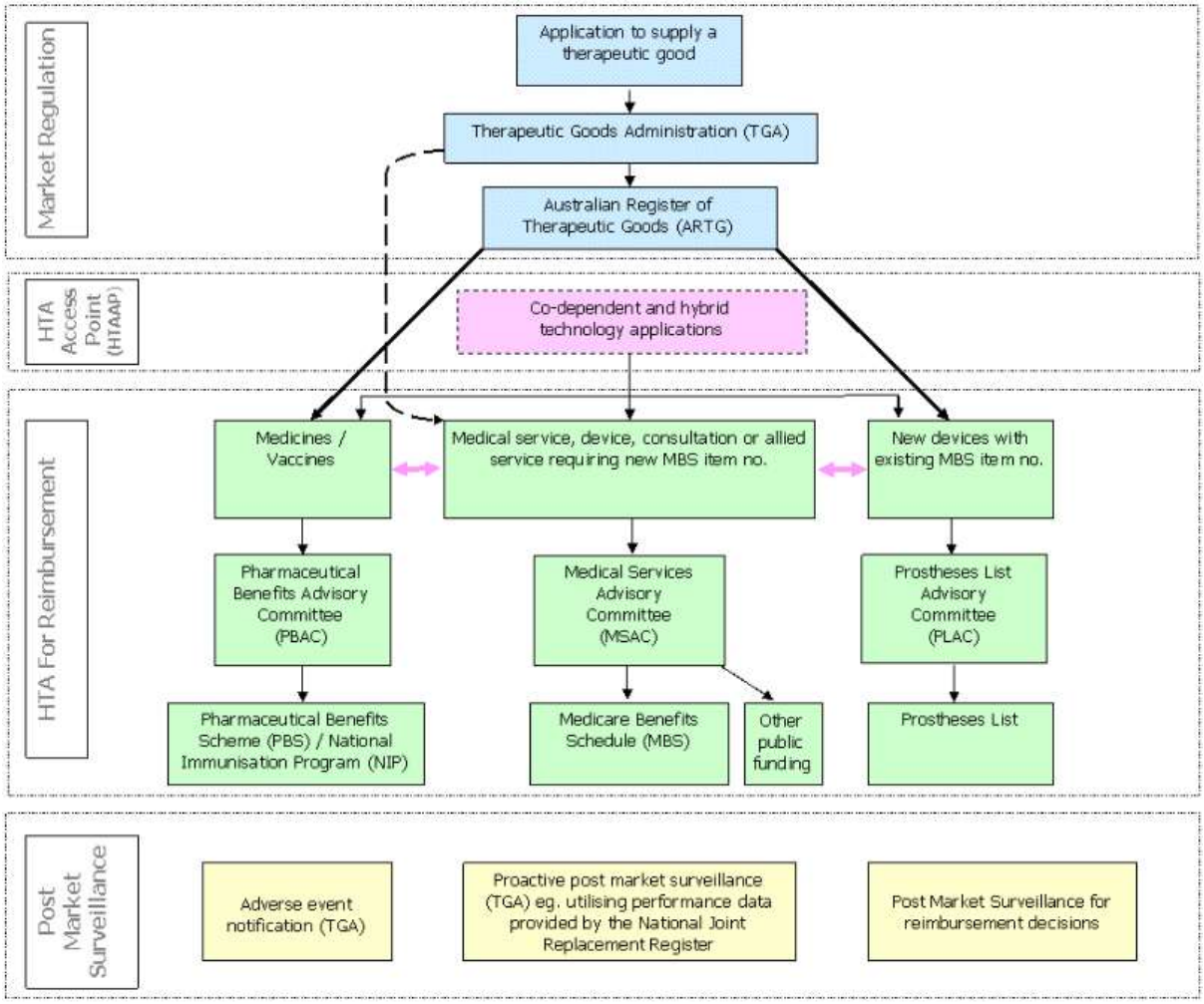
148 For the principles and methodologies used by the PBAC, see: DoH, *Guidelines for preparing a submission to the Pharmaceutical Benefits Advisory Committee*, September 2016, p. 4.

149 *National Health Act 1953*, s. 100A.

150 Professor Wilson, PBAC, *Committee Hansard*, 29 August 2017, p. 10.

151 Professor Wilson, PBAC, *Committee Hansard*, 29 August 2017, p. 10.

Figure 4: Map of current Australian Government HTA processes for market entry and for reimbursement processes[152]



2.108 On 24 October 2014, the Australian government announced an independent review of the regulation of medicines and medical devices (MMDR review) to:

> …identify ways to assist medicine and medical device producers and suppliers struggling with complex and costly regulatory pathways, while upholding the safety and efficacy of therapeutic goods available in Australia.[153]

2.109 The 58 recommendations of the review were published in July 2015, and included:

- expanding the pathways by which sponsors can seek marketing approval for a medicine or medical device, including making provision for utilisation of

152 DoH, *Health Technology Assessment (HTA) overview*, 7 March 2017 (accessed 1 November 2017).

153 The Hon. Peter Dutton, MP, Minister for Health and Senator The Hon Fiona Nash, Assistant Minister for Health, 'Expert Panel to Review Medicines and Medical Devices Regulation', *Media Release*, 24 October 2014.

assessments conducted by comparable regulators, and for expedited assessments in defined circumstances;

- identifying comparable overseas national regulator authorities using transparent criteria;
- enhancing post-market monitoring of medicines and medical devices and streamline post-market requirements in respect of products in the Australian Register of Therapeutic Goods; and
- improving transparency and predictability of processes and decisions to build trust and confidence in the Australian National Regulatory Authority's ability to ensure Australians have timely access to high quality, safe and efficacious products.[154]

2.110 The Australian government released its response to the MMDR review on 15 September 2016, and noted that the expert panel conducting the MMDR review:

> …provided a strong case for the reform of the regulation of therapeutic goods in Australia - one that strikes a balance between supporting consumer choice, the safe and effective use of therapeutic products, creates flexibility for industry and ensures that regulatory settings are appropriately aligned to risk.[155]

2.111 The government noted its intention to implement the majority of recommendations arising from the MMDR review:

> …in a staged approach over the next three years in order to maintain continuity of business. The Department of Health will collaborate and consult across government and with consumers, health professionals and industry in order to progress these reforms. The TGA, where necessary, will cost recover from industry so as to ensure that it is adequately resourced to implement these reforms and undertake the ongoing work without interrupting business as usual.
>
> The Government understands that consumer, professional, and industry groups are looking for immediate action. Accordingly, the Department of Health will commence work on designing implementation of the recommendations, with a view to implementing early opportunities in 2016-2017. Implementation of this important programme of reform will deliver significant benefits for the Australian public and to the Australian medicine and medical device industries.[156]

2.112 The government also recognised several benefits of its approach, including:

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154 Expert Panel Review of Medicines and Medical Devices Regulation, *Recommendations to the Minister for Health on the Regulatory Frameworks for Medicines, Medical Devices, Complementary Medicines and Advertising of Therapeutic Goods*, 31 July 2015.

155 Australian government, *Australian Government Response to the Review of Medicines and Medical Devices Regulation*, 15 September 2016, p. 4.

156 Australian government, *Australian Government Response to the Review of Medicines and Medical Devices Regulation*, 15 September 2016, p. 5.

- access to life-saving and innovative medicines and medical devices will be improved through the introduction of new, expedited pathways for approval. This will lead to earlier access to vital, life-saving therapies for patients with serious conditions;
- faster access for Australian consumers to certain medicines and medical devices that are approved based on assessments from comparable overseas regulators. This will reduce duplication of effort, leading to efficiencies, while ensuring Australian consumer protection is maintained through retention of oversight by the TGA as the final decision-making authority;
- consumer protection will be enhanced through the development of a more comprehensive system of post-market monitoring which will provide the TGA with better information about emerging safety issues. This will ensure that therapeutic goods in Australia continue to be safe for use, efficacious and of a good quality.[157]

2.113 The TGA website notes that the government has been consulting internally, with the public, and with particular stakeholders on the implementation of the accepted recommendations arising from the review,[158] and states that some of the reforms 'require changes to legislation':

> This large program of work was divided into two tranches; the first set of legislative changes were passed 14 June 2017. These focused on new assessment pathways for medicines and medical devices. The second tranche of legislative review is underway. The progress of these amendments may influence the timing of some regulatory changes.[159]

2.114 The reforms already implemented are:

- those made to category C of the Special Assistance Scheme, namely, the '[i]mplementation of a notification scheme rather than pre-approval for supply of certain unapproved therapeutic goods to patients';[160] and
- the priority review pathway for prescription medicines, which 'will involve faster assessment of vital and life-saving prescription medicines for which a complete data dossier is available' within 150 working days, which is 'up to

157 Australian government, *Australian Government Response to the Review of Medicines and Medical Devices Regulation*, 15 September 2016, p. 6.

158 Therapeutic Goods Administration (TGA), *Medicines and medical devices regulation review*, 28 August 2017, https://www.tga.gov.au/mmdr (accessed 13 November 2017).

159 TGA, *Medicines and medical devices regulation review*, 28 August 2017, https://www.tga.gov.au/mmdr (accessed 13 November 2017).

160 TGA, *Medicines and medical devices regulation review*, 28 August 2017, https://www.tga.gov.au/mmdr (accessed 13 November 2017).

> three months shorter than the standard prescription medicines registration process'.[161]

2.115 As indicated above, the TGA is looking to implement a number of other measures, such as the 'provisional approval pathway' which:

> …will provide earlier access to certain promising new medicines that do not yet have a full dossier of clinical data, but where there is the potential for a substantial benefit to Australian patients through the earlier availability of these medicines.[162]

2.116 In September 2015, the Senate Community Affairs References Committee (Community Affairs Committee) reported on the effectiveness of the HTA process in respect of the availability of new, innovative and specialist cancer drugs in Australia.[163] The Community Affairs Committee urged the government 'to give careful consideration to the implementation' of the recommendations made as a result of the MMDR review[164] and made three key recommendations in its report, namely that the Australian government:

- initiate a comprehensive review of the system for the registration and subsidisation of medicines, setting out what types of factors should be examined;
- commission a review of current data collection mechanisms for cancer medicines, providing examples of factors to be included in the review; and
- establish a Steering Committee to examine the feasibility of establishing a national register of cancer medicines.[165]

2.117 The government has recently responded to the Community Affairs Committee report, in which it supported the intent of the first and second recommendations, and did not agree to the third. In its response, the government outlined the work it is already undertaking in response to the MMDR review. For example, it highlighted that:

> Patients and sponsors will benefit from two expedited pathways being implemented by the TGA, which will help to achieve earlier regulatory approvals of new life-saving medicines such as new cancer medicines, or to extend uses of existing medicines to treat a new population of patients (for

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161 TGA, *Priority review pathway: prescription medicines*, 26 June 2017, https://www.tga.gov.au/priority-review-pathway-prescription-medicines (accessed 13 November 2017).

162 TGA, *Medicines and medical devices regulation review*, 28 August 2017, https://www.tga.gov.au/mmdr (accessed 13 November 2017).

163 Senate Community Affairs References Committee, *Availability of new, innovative and specialist cancer drugs in Australia*, September 2015.

164 Senate Community Affairs References Committee, *Availability of new, innovative and specialist cancer drugs in Australia*, September 2015, pp 109–110.

165 Senate Community Affairs References Committee, *Availability of new, innovative and specialist cancer drugs in Australia*, September 2015, pp 110–112.

> example, a treatment already approved for one type of cancer being used to treat another type of cancer).[166]

2.118 The government recognised that, although the MMDR review 'did not include consideration of PBS listing and PBAC processes' the implementation processes in response to the review will impact on these processes.[167]

2.119 The government also referred to consultation with industry that is on foot with regard to:

> … a pilot project involving a joint TGA/PBAC pre-submission meeting, use of a single clinical evaluation report that meets both regulatory and reimbursement authority requirements, and information sharing post-market monitoring.[168]

2.120 Professor R John Simes advocated for further interconnectedness between these individual mechanisms of the HTA process, namely between government funding sources and the PBAC and MSAC. Professor Simes called for bodies such as the MRFF to broaden their criteria for funding to include return on investment, which he argued should also be linked to the PBAC and MSAC, as:

> …if you have a drug which is supported through the PBS, there is evidence for it. If the evidence does not exist, you cannot get funding for that particular drug through the PBS; there is not a mechanism to do so.[169]

2.121 Further discussion about the PBAC and MSAC, and how their processes affect LSR cancers, appears at chapter 5.

2.122 Another issue raised with the committee with respect to the HTA process is the delay from registration by the TGA to listing on the PBS. For example, Medicines Australia referred to its earlier submission to the Community Affairs Committee inquiry, where it identified that this process, on average, takes 'in excess of 18 months', and further noted:

- New listings take on average 589 days (over 1 ½ years)
- Subsequent listings take on average 700 days (nearly 2 years)
- Disturbingly, some medicines took up to 1,600 days (4 ½ years) for a new listing and 2,400 days (more than 6 ½ years) for a subsequent listing.[170]

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166 Australian government, *Australian Government response to the Senate Community Affairs References Committee Report: Availability of new, innovative and specialist cancer drugs in Australia*, November 2017, p. 6.

167 Australian government, *Australian Government response to the Senate Community Affairs References Committee Report: Availability of new, innovative and specialist cancer drugs in Australia*, November 2017, p. 7.

168 Australian government, *Australian Government response to the Senate Community Affairs References Committee Report: Availability of new, innovative and specialist cancer drugs in Australia*, November 2017, p. 9.

169 Professor R John Simes, Executive Member, Cooperative Trials Group for Neuro-Oncology; and Director, NHMRC Clinical Trials Centre, University of Sydney, *Committee Hansard*, 18 May 2017, p. 53.

2.123 More recently, Medicines Australia commissioned a Deloitte report which detailed the duration taken in the HTA process for certain cancer medicines during the period 2010–2016:

Table 4: Number of months to events in the PBS process for 147 'high level' submission for cancer medicines (2010-2016)[171]

Time-to-event analysis	Overall	New cancer medicine	New cancer indication
Period from date of initial PBAC submission to date of PBS listing (months)*	20.5 (49)	20.9 (24)	20.1 (25)
Period from date of initial PBAC submission to date of last PBAC outcome (months)*	11.6 (90)	12.3 (47)	10.9 (43)
Period from date of TGA registration to date of PBS listing (months)	22.2 (49)	18.6 (24)	25.7 (25)
Period from date of PBAC recommendation to date of PBS listing (months)	7.4 (49)	7.6 (24)	7.3 (25)

Source: Wonder Drug Consulting, October 2016, Analysis of PBAC submissions and outcomes for medicines for patients with cancer (2010-2016)

'High level' submissions mean submissions for new medicines (i.e. new listings) and new indications (i.e. new use within a given cancer, irrespective of PBAC major or minor submissions.

Numbers in parentheses are the sample sizes

2.124 Medicines Australia also provided the committee with a comparison of the Australian reimbursement system with those of other OECD countries which appears at Figure 5—where Australia ranks 18th out of 20 countries, ahead of Portugal and New Zealand—also noting that 'of all the new medicines registered by the TGA between 2009 and 2014, only 39 per cent of them were reimbursed in Australia'.[172]

170 Medicines Australia, Submission 142 to the Senate Community Affairs References Committee, *Availability of new, innovative and specialist cancer drugs in Australia*, September 2015, p. 14 (citations omitted).

171 Deloitte, *A Collaborative Assessment of Access to Cancer Medicines in Australia*, May 2017, p. 16.

172 Medicines Australia, answers to questions on notice, 8 June 2017, (received 20 October 2017), p. 8.

Figure 5: Proportion of registered medicine which eventually secured reimbursement—by country—2009 to 20014[173]

2.125 Indeed, the Community Affairs Committee outlined in its report that a key factor that affects access to medicines, 'is the timing of applications by pharmaceutical companies to the TGA seeking registration of medicines and to the PBAC seeking reimbursement'.[174] Further:

> The Department of Health (DOH) noted that for cancer medicines submitted for TGA approval between 2009-2014, submissions were made an average of 38 weeks after the lodgement of a submission to the [US] Food and Drug Administration (FDA) and an average of 38 weeks after the lodgement of a submission to the European Medicines Agency (EMA). DOH told the committee that this approach is often a function of the size of the Australian market:
>
> > This kind of business approach seeks to establish, as early as possible, a positive response in the regions offering the most potential for profit, due to their large population size. This avoids the situation where a deferral or rejection from a country with a small population, like Australia, could influence other authorities, thereby jeopardising the profit margins that could be achieved in larger countries/regions.[175]

2.126 The Community Affairs Committee acknowledged that the DoH's evidence illustrated that 'this factor is outside the control of the TGA and PBAC', and also cited

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173 Medicines Australia, answers to questions on notice, 8 June 2017, (received 20 October 2017), p. 9.

174 Senate Community Affairs References Committee, *Availability of new, innovative and specialist cancer drugs in Australia*, September 2015, p. 17.

175 Senate Community Affairs References Committee, *Availability of new, innovative and specialist cancer drugs in Australia*, September 2015, p. 17 (citations omitted).

evidence from the DoH that '[t]he ability to deliver timely access to medicines is also affected by the timing of the applications which, in Australia, is at the discretion of pharmaceutical companies' that may choose to apply for approval in the US or Europe ahead of Australia.[176]

2.127 In terms of developments in the US, the committee also heard that the FDA had recently approved, for the first time, a drug based on the molecular profile of a tumour, rather than its location:

> The FDA approved the first drug just a couple of weeks ago, Keytruda, which is for any cancer types from anywhere in the body which is mismatch repair deficient tumours. There is a big shift. So pharma companies are starting to see this shift as well and look at drugs across tumour types. From the perspective of genomics, we already think like that.[177]

2.128 Subsequently, in August 2017, the FDA made a comparable ruling on an immunocellular therapy, which Deloitte described as 'signalling its commitment to modernising its processes in alignment with the therapeutic landscape'.[178]

2.129 However, Professor Wilson considered that a lot of research into cell biology is 'very basic research' that will take 'many, many years' to reach fruition.[179]

Current funding for LSR cancers

2.130 Despite accounting for five times the number of other cancer deaths in Australia, rare cancers receive just $6 million annually in NHMRC funding.[180] This can be seen in Figure 6, which illustrates the total amount of funding, including NHMRC funding, awarded to research into cancers from 2006–2011, compared to mortality rates for these cancers.

176 Senate Community Affairs References Committee, *Availability of new, innovative and specialist cancer drugs in Australia*, September 2015, pp 17–18 (citations omitted).

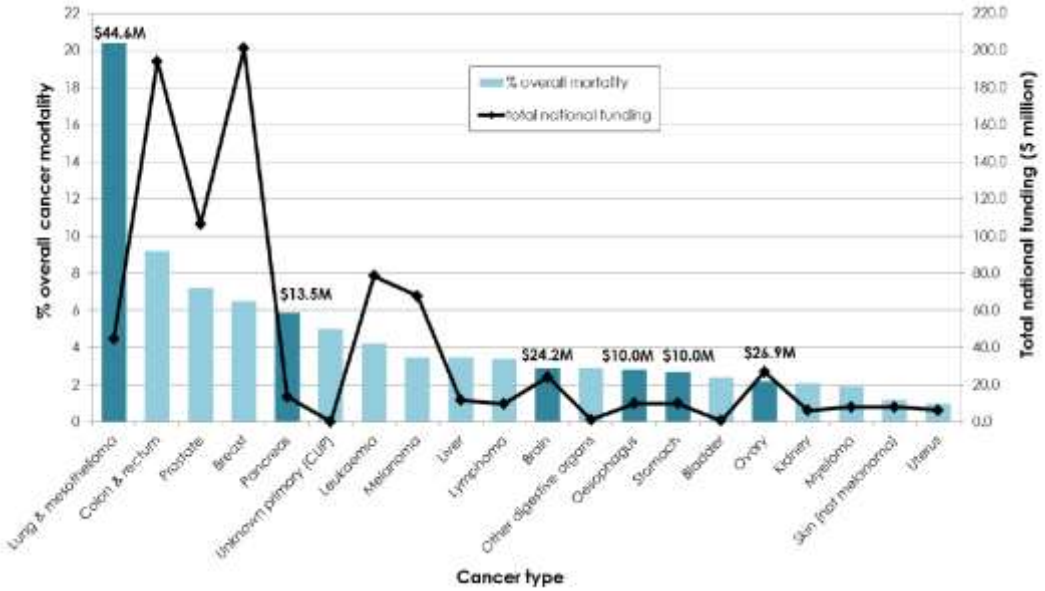
177 Dr Nicola Waddell, Group Leader, Medical Genomics Group, QIMR Berghofer, *Committee Hansard*, 6 June 2017, p. 45.

178 Deloitte, *The New Wave of Immunotherapy Cancer Medicines –The Untapped Potential for Australians*, October 2017, p. 51.

179 Professor Wilson, PBAC, *Committee Hansard*, 29 August 2017, p. 12.

180 Professor Thomas, Garvan Institute, *Committee Hansard*, 8 June 2017, p. 31.

Figure 6: National funding to cancer type-specific research in Australia (2006–2011) compared with the top 20 cancers by overall cancer mortality (2012)[181]



2.131 This information, and an in-depth analysis of major government and non-government funding of cancer research in Australia appears in Cancer Australia's 2015 publication *Cancer Research in Australia: an overview of funding initiatives to support cancer research capacity in Australia 2006 to 2011*, which is the 'first national overview of funding to cancer research in Australia'.[182]

2.132 Consistent with the discussion at paragraphs 2.4–2.8 about funding into cancer research during the period 2016–2018, Figure 7 illustrates that in 2006–2011 the Australian government was the 'major funder of cancer research projects and research programs, people support scheme awards, and building cancer research capacity initiatives and infrastructure awards' providing 58 per cent, or $1.03 billion, of funding.[183]

2.133 As can be seen, 43 per cent of this funding came via the NHMRC with 15 per cent coming from other sources such as the Department of Industry (including the Australian Research Council), Cancer Australia and the DoH.[184]

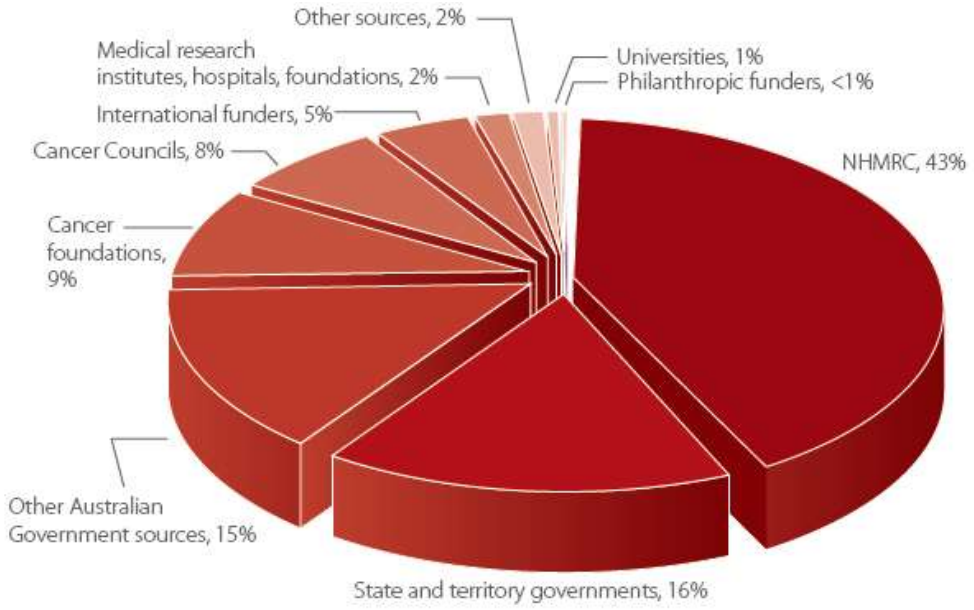
181 Cancer Australia, *Submission 129*, p. 5. This excludes data for acute myeloid leukaemia, which was not available.

182 Cancer Australia, *Submission 129*, p. 4.

183 Cancer Australia, *Cancer Research in Australia: an overview of funding initiatives to support cancer research capacity in Australia 2006 to 2011*, 2015, p. 112.

184 Cancer Australia, *Cancer Research in Australia: an overview of funding initiatives to support cancer research capacity in Australia 2006 to 2011*, 2015, p. 112.

Figure 7: Proportion of funding by funding source to cancer research projects and research programs, building cancer research capacity initiatives, and infrastructure awards[185]



2.134 Despite this seemingly large allocation of government funding for cancer research, the committee received a number of submissions[186] and heard from a number of witnesses[187] who criticised the lack of government funding for research into LSR cancers.

2.135 For example, the CBCF submitted that the government's current use of the burden of disease approach to assess the prioritisation and funding in respect of cancer is 'no longer an appropriate measure to use' to make this assessment, as the use of the 'disability-adjusted life years' (DALYs) model:

> …lost appropriateness when five-year survival for higher incidence, and comparatively well-funded, cancers (e.g. breast, prostate and childhood leukaemia) started to get close to 100% in stark contrast to other (far) lower-survival and (considerably) lower-funded cancers.[188]

185 Cancer Australia, *Cancer Research in Australia: an overview of funding initiatives to support cancer research capacity in Australia 2006 to 2011*, 2015, p. 113.

186 See, for example, Ms Christine Jones, *Submission 6*; Asbestos Council of Victoria/GARDS, *Submission 30*; Mrs Madeline Bishop, *Submission 35*.

187 See, for example, Mrs Sandra Joy Woods, *Committee Hansard*, 18 May 2017, p. 5; Mrs Nicole Mills, Executive Officer, Rare Voices Australia, *Committee Hansard*, 7 June 2017, p. 26; Mrs Berman, BTAA, *Committee Hansard*, 8 June 2017, p. 39.

188 CBCF, *Submission 139*, p. 6.

2.136 A DALY measures the 'disease burden and combines data on the extent of premature death and non-fatal health impacts of disease'.[189] Using this measure as a reference for health expenditure, Cancer Australia outlined that it was:

> …estimated that in 2012, cancer caused 551,300 DALYs to be lost, representing 19% of the burden of all diseases in Australia. By comparison, cardiovascular disease contributed to 16% of the burden of disease, whilst nervous system and sense organ disorders accounted for 14% of the burden of disease and mental disorders accounted for 13% of the burden of disease. In terms of health care expenditure, in 2008–09, cancer and other neoplasms accounted for $5 billion or 7% of total recurrent health spending.[190]

2.137 The AIHW informed the committee that in addition to DALYs, 'quality-adjusted life years' (QALYs) can be used as 'a measure of potential health gain from the effect of interventions'.[191] Therefore, both DALYs and QALYs can 'be used in health economic evaluations as a measure of health gain to estimate the potential health benefits of specific health interventions'.[192] However, the AIHW noted that the 'DALY is the standard measure used in burden of disease studies'.[193]

2.138 Another criticism of the lack of funding into LSR cancers was raised by Ms Elizabeth de Somer of Medicines Australia, who commented that although there had been some welcome steps, including the announcement of the first MRFF disbursements, 'there is nothing that particularly targets the rare and low-survival cancers'.[194]

2.139 Indeed, the CBCF stated in its submission that LSR cancers, including brain cancer, 'have been for some time, in effect discriminated against, within the Government funding system'.[195] The CBCF submitted that LSR cancers 'are clearly unmet medical needs which should be afforded special status by earmarking specific funds and prioritising focus around them'.[196]

2.140 Mrs Evangeline Lim, diagnosed with advanced lung cancer in November 2016, described the personal impact of this lack of funding:

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189 Cancer Australia, *Cancer Research in Australia: an overview of funding initiatives to support cancer research capacity in Australia 2006 to 2011*, 2015, p. 19.

190 Cancer Australia, *Cancer Research in Australia: an overview of funding initiatives to support cancer research capacity in Australia 2006 to 2011*, 2015, p. 19.

191 AIHW, answers to questions on notice, 8 June 2017, (received 3 July 2017), p. 11.

192 AIHW, answers to questions on notice, 8 June 2017, (received 3 July 2017), p. 11.

193 AIHW, answers to questions on notice, 8 June 2017, (received 3 July 2017), p. 12.

194 Ms Elizabeth de Somer, Director of Policy and Research, Medicines Australia, *Committee Hansard*, 8 June 2017, p. 21.

195 CBCF, *Submission 139*, p. 6.

196 CBCF, *Submission 139*, p. 6.

> I am sad with the injustice of research funding allocated to lung cancer. We only get less than five cents in cancer research funding, and lung cancer has a 15 per cent survival rate of living for five years from diagnosis.[197]

2.141 Following the due date for submissions and before the committee's final hearing, on 24 August 2017, the government announced $13 million of funding for competitive research grants from the MRFF, 'designed to boost clinical trial registry activity with priority given to under-researched health priorities, such as rare cancers and rare diseases'.[198]

2.142 The desired outcomes of this investment are as follows:

- New opportunities for those suffering from rare cancers and rare diseases to participate and benefit from the latest research.
- Attention given to under researched health priorities and conditions.
- Deployment of innovative trial designs and recruitment strategies.
- Purposeful health service engagement to improve the translation of research into practice and improve outcomes for patients.
- New health treatments, drugs and devices to improve health.
- Reinforcement of Australia's position as a preferred destination for clinical trials.[199]

2.143 The DoH subsequently provided information to the committee that, from 2013–14 to 2016–17 it provided approximately $9.1 billion for cancer services and research, which is exclusive of funding from portfolio agencies, such as the NHMRC and Cancer Australia.[200]

2.144 In evidence to the committee on 29 August 2017, the DoH identified several of the MRFF programs that are underway under the trials and translation platform:

> Lifting clinical trials and registries capacity, clinical trials networks, has $5 million allocated to it. Trial activities specifically targeting adolescents and young adults living with cancer has $5 million of funding for CanTeen. Lifting clinical trials and registries capacity research grants has $13 million, which is designed to accommodate clinical trials on rare cancers and rare diseases. Eight million dollars has been allocated to the next generation of clinical researchers.[201]

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197 Mrs Evangeline Lim, *Committee Hansard*, 6 June 2017, p. 3.

198 The Hon. Greg Hunt MP, 'Major new measures to help combat rare cancers', *Media Release*, 24 August 2017, p. 1.

199 DoH, *Rare Cancers and Rare Diseases - Research Grants*, 24 August 2017, http://www.health.gov.au/internet/main/publishing.nsf/Content/mrff-rare-cancers-rare-diseases-grants-2017 (accessed 11 October 2017).

200 DoH, answers to questions on notice, 29 August 2017, (received 22 September 2017).

201 Mr Nicholas Hartland, First Assistant Secretary, Research, Data and Evaluation, DoH, *Committee Hansard*, 29 August 2017, p. 9.

2.145 The DoH also informed the committee of MRFF investments that are specifically relevant to rare cancers:

> In the first disbursements under the MRFF, which were announced in the context of the 2017-18 budget, $69.5 million was dispersed from the fund. There are a couple of relevant initiatives, particularly related to clinical trials. One is an investment in clinical trial networks, which are often perceived to be the backbone of the trial industry in Australia. They support investigator-driven activity. They answer questions of service delivery and comparative effectiveness. And we have funded $5 million—the Australian Clinical Trial Alliance— to lift the capacity of these networks that occur across a number of specialties. That's in the process of being ramped up
>
> We also invested $5 million through CanTeen to target trial activity for adolescents and young adults. This cohort sometimes has difficulty gaining access to trials—caught between kids and adults. That activity has been executed. CanTeen is progressing with that work. Last Thursday, 24 August, the minister announced the opening of a $13 million clinical trial and registry program. It's actually titled Lifting Clinical Trials and Registries Capacity. This is directly relevant to the committee because it is designed to attract activity that addresses burden and unmet need. By that I mean rare cancers and rare diseases. In fact, the guidelines preference rare cancer and rare disease applicants. It also is looking at innovative trial methodologies, like, for example, adaptive trial platforms, some innovative and novel approaches to doing trial activity and the application of precision medicine in a trial environment, which is increasingly being used to do a sequence of an individual and specifically target the treatment to that patient. For lots of different reasons, it is beneficial and, perhaps some would argue, even cost effective.
>
> Then of course, there is investment in researchers, because you can't just inject a whole bunch of money into the system without building the capacity of researchers. So $8 million to top up existing NHMRC medical practitioner fellowships—and that's progressing quite well as well too. So I think those programs are a demonstration of the sorts of things that you may see over time from the MRFF.[202]

2.146 The DoH also highlighted a number of features in its *Medical Research Future Fund - Lifting Clinical Trials and Registries Capacity (LCTRC) Grant Guidelines* that it considered relevant to the committee's terms of reference:

> The assessment criteria are slightly different to traditional clinical trial structures, so they're divided into three sections. Forty per cent is for significance of grant outcomes, another 40 per cent is for scientific quality and 20 per cent is weighted for team quality and capacity. I think that allocation of 40 per cent for significant grant outcomes presents a lot of opportunity for researchers who, in the space of rare cancers and low-survival cancers, may not have the track record of other researchers. What

202 Ms Erica Kneipp, Assistant Secretary, DoH, *Committee Hansard*, 29 August 2017, p. 11.

> we're hoping to do with that weighting is also to generate some innovative ideas and design approaches to trials through this application round.[203]

2.147 The significance of the grant outcome is defined in the Guidelines, where '[s]ignificance is the potential to increase knowledge of important topics that achieve the outcomes of the grant opportunity', and will be assessed by reference to a number of considerations.[204]

Quarantining funding

2.148 A number of submitters and witnesses advocated for specific funding to be set aside for research into low survival rate cancers.[205]

2.149 In respect of quarantining NHMRC funding, Professor Kelso considered that the NHMRC's current model of funding is appropriate, especially in light of the priority-driven funding offered by the MRFF.[206]

2.150 This was reflected in the evidence of Associate Professor Wright, who opined that quarantined funding 'could specifically target that preliminary research that is required to build track record and eventually produce a successful funding application', and suggested that such funding could come from the MRFF:

> I am suggesting that the NHMRC as it stands supports 13 per cent of fundable research—that is very high-quality research. I have reviewed that sort of research as part of my job as a researcher. I have reviewed other people's grants, and I have seen grants that I think must get funded but that do not get funded, just because there are not enough funds in the pool. It is not because of any bias; it is just that that is the pool of money, that is how much good research is being put forward, and that is how much preliminary work has been done. Huge amounts of money and time have been put into those applications, to go nowhere, or it has rolled over to next year. So it has to be from outside the NHMRC. You cannot divide up the pie anymore. That is why, if we have a new source such as the MRFF, I would say that is where that sort of funding clearly has to come from, or it is an example of where it should come from. I am just saying it should not come out of NHMRC.[207]

2.151 Dr Robert De Rose, who noted that the MRFF research parities had been set for the next two years, suggested that the review of the MRFF priorities in 2018 would be:

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203 Ms Kneipp, DoH, *Committee Hansard*, 29 August 2017, p. 11.

204 DoH, *Medical Research Future Fund - Lifting Clinical Trials and Registries Capacity (LCTRC) Grant Guidelines*, 24 August 2017, pp 13–17 (tabled 29 August 2017).

205 See, for example, The Unicorn Foundation, *Submission 101*, p. 5; The University of Newcastle and Hunter Medical Research Institute, *Submission 132*, p. 2; Professor Thomas, Garvan Institute, *Committee Hansard*, 8 June 2017, p. 36.

206 Professor Kelso, NHMRC, *Committee Hansard*, 29 August 2017, p. 27.

207 Associate Professor Wright, VCCC, *Committee Hansard*, 7 June 2017, pp 36–37.

> …an opportunity to address the funding shortfall for cancers with low survival rates. We cannot just repeat the consultation process that was used last year to allocate funding for the first two years. This will likely result in a similar outcome. A small amount of the research allocation should be prioritised for low-survival cancers. Otherwise, the current stakeholders will win out.[208]

2.152 In responding to the question of quarantining MRFF funding, Professor Frazer noted that the powers to allocate funding are vested in the minister,[209] pointing out that the AMRAB advises the minister about how to allocate funding, but that 'he is not required to follow our advice'.[210] Professor Frazer noted that the AMRAB had also recommended, going forward, that 'the grants given out should be longer term and larger scale project grants of the order of five years' in order to 'allow bigger problems, if you like, and problems which require more effort over a longer period of time for a larger number of people to be contemplated'.[211]

Committee view

2.153 It is apparent to the committee that there is an inadequate amount of government and non-government funding allocated towards research into LSR cancers.

2.154 The committee agrees with evidence it has received which demonstrates that the rate of survival for people with LSR cancers will remain stagnant until significantly more funding is allocated for research into these cancers.

2.155 The committee acknowledges the finite amount of government money available for all forms of medical research, and therefore welcomes the government's recent announcement of $13 million of funding for competitive research grants from the MRFF that will prioritise 'under-researched health priorities, such as rare cancers and rare diseases'.[212] It also welcomes the more recent announcement, on 29 October 2017 of the Australian Brain Cancer Mission, a $100 million collaboration of the Australian government, the CBCF and philanthropy to defeat brain cancer.[213]

2.156 The prioritisation of rare cancers and rare diseases in the granting of this funding suggests that the government acknowledges the importance of allocating discrete amounts of funding in order to make progress in combatting rare cancers and rare diseases.

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208 Dr Robert De Rose, Co-founder, The Isabella and Marcus Fund, *Committee Hansard*, 7 June 2017, p. 58.

209 Pursuant to s. 15A of the *Medical Research Future Fund Act 2015*.

210 Professor Frazer, AMRAB, *Committee Hansard*, 8 June 2017, pp 48–49.

211 Professor Frazer, AMRAB, *Committee Hansard*, 8 June 2017, p. 49.

212 The Hon. Greg Hunt MP, 'Major new measures to help combat rare cancers', *Media Release*, 24 August 2017, p. 1.

213 The Hon. Greg Hunt MP, 'Australian Brain Cancer Mission', *Media Release*, 29 October 2017.

2.157 However, the committee considers that, in order to effectively increase survival rates for people with LSR cancers, the government should go further and, as some submitters and witnesses have suggested, guarantee government funding specifically for research into LSR cancers.

2.158 The committee acknowledges that the NHMRC Act prohibits the minister from recommending 'the allocation of research funds to a particular person, organisation, State or Territory';[214] however, the Act also empowers the CEO of the NHMRC to identify National Health Priority Areas (NHPAs): major national health issues that make a significant contribution to the burden of disease[215] to which a 'substantial proportion of NHMRC funding is directed'.[216] 'Cancer control' is one of the NHPAs in the NHMRC's Corporate Plan 2017–18.[217]

2.159 The committee urges the CEO of the NHMRC to consider identifying LSR cancers as a NHPA in the upcoming 2018–19 Corporate Plan. The minister may be able to require the NHMRC to do so by way of a referral, pursuant to section 5D of the NHMRC Act, or a ministerial direction, pursuant to section 5E of the NHMRC Act.

Recommendation 1

2.160 The committee recommends that the Chief Executive Officer of the National Health and Medical Research Council considers identifying low survival rate cancers as a National Health Priority Area in the upcoming 2018-19 Corporate Plan.

2.161 The committee welcomes NHMRC's recent restructure of its grants program. In particular, it supports the introduction of the Ideas Grant scheme which will encourage innovation and assist early-career researchers launch their careers. The committee considers that it is important to encourage researchers to work on LSR cancers as this will also contribute to increased survival rates for people with these cancers.

2.162 Further, the committee considers that the extension of the duration of NHMRC grants—to five years for the duration of the Investigator Grants and Synergy Grants and up to five years for the Ideas Grants—demonstrates the NHMRC's understanding of the long time required to conduct medical research and obtain meaningful results.

2.163 However, the committee is disturbed by the evidence that some drugs may take 10 to 15 years to develop—much longer than a 5 year grant— and that some

214 *National Health and Medical Research Council Act 1992*, s 5D.

215 NHMRC, *Major health issues*, https://www.nhmrc.gov.au/book/nhmrc-corporate-plan-2016-2017/nhmrc-s-strategic-direction/major-health-issues (accessed 22 November2017).

216 NHMRC, *National Health and Medical Research Council Corporate Plan 2017-18*, https://www.nhmrc.gov.au/_files_nhmrc/file/grants/apply/17293_nhmrc_corporate_plan_2017-18-web.pdf (accessed 22 November 2017), p. 18.

217 NHMRC, *National Health and Medical Research Council Corporate Plan 2017-18*, p. 18.

research is abandoned when funding is no longer available. For these reasons, the committee recommends that the NHMRC introduces the option for extensions to the duration of grants, provided that recipients satisfy certain performance criteria.

Recommendation 2

2.164 The committee recommends that the National Health and Medical Research Council introduces the option for extensions to the duration of funding to recipients of research grants, provided that these recipients satisfy certain performance criteria.