The Senate

Select Committee into Funding for Research into Cancers with Low Survival Rates

Report

November 2017

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Members of the committee

Members

Senator Catryna Bilyk (ALP, TAS) (Chair)

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Ms Sophie Dunstone, Committee Secretary

Miss Jedidiah Reardon, Principal Research Officer

Mr Nicholas Craft, Senior Research Officer

Ms Nicola Knackstredt, Senior Research Officer

Ms Sophie Clarke, Administrative Officer

Ms Jo-Anne Holmes, Administrative Officer

PO Box 6100
Parliament House
CANBERRA ACT 2600

Telephone: (02) 6277 3419
Fax: (02) 6277 5809
Email: cancer.research.sen@aph.gov.au
Internet: http://www.aph.gov.au/senate_cancerresearch

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Abbreviations

AGITG	Australasian Gastro-Intestinal Trials Group
AHES	Australian Health Economics Society
AIHW	Australian Institute of Health and Welfare
ALK	anaplastic lymphoma kinase
ALL	acute lymphoblastic leukaemia
ALLG	Australasian Leukaemia and Lymphoma Group
AMRAB	Australian Medical Research Advisory Board
ANZCHOG	Australian and New Zealand Children's Haematology and Oncology Group
AYAs	adolescents and young adults
BCDC	Brain Cancer Discovery Collective
BMS	Bristol-Myers Squibb
BTAA	Brain Tumour Alliance Australia
CBCF	Cure Brain Cancer Foundation
CCA	Cancer Council Australia
CCDR	Centre for Community-Driven Research
CCRU	Children's Cancer Research Unit
CCV	Cancer Council Victoria
CEO	Chief Executive Officer
CFDA	Chinese Food and Drug Administration
COAG	Council of Australian Governments
COG	Children's Oncology Group
COSA	Clinical Oncology Society of Australia
CPD	continuing professional development
CPR	cardio-pulmonary resuscitation
CSO	Common Scientific Outline

CTA	Clinical Trial Applications
CVA	Cancer Voices Australia
DALYs	disability-adjusted life years
Deloitte	Deloitte Access Economics
DIPG	diffuse intrinsic pontine glioma
DoH	Department of Health
DOH	United States Department of Health
DSP	Disability Support Pension
DSS	Department of Social Services
EMA	European Medicines Agency
FDA	Food and Drug Administration
Garvan Institute	Garvan Institute of Medical Research
GCMP	Genomic Cancer Medicine Program
GIST	Gastro Intestinal Stromal Tumour
GPs	general practitioners
HREC	Human Research Ethics Committee
HTA	Health Technology Assessment
ICGC	International Cancer Genome Consortium
ICGCmed	ICGC medicine
KPIs	Key Performance Indicators
LCSA	Low Cancer Survivals Alliance
LCTRC	Lifting Clinical Trials and Registries Capacity
LFA	Lung Foundation Australia
LSR	low survival rate
MBS	Medicare Benefits Schedule
MDTs	Multi-Disciplinary Teams
MHF	Mark Hughes Foundation

MMDR review	independent review of the regulation of medicines and medical devices
MoST	Molecular Screening and Therapeutics
MRFF	Medical Research Future Fund
MRFF Act	*Medical Research Future Fund Act 2015*
MSAC	Medical Services Advisory Committee
MSD	Merck Sharp & Dohme Australia
NCERG	National Cancer Expert Reference Group
NCI	National Cancer Institute
NEAF	National Ethics Application Form
NET	neuroendocrine cancer/ neuroendocrine tumour
NHMRC	National Health and Medical Research Council
NHMRC Act	*National Health and Medical Research Council Act 1992*
NHPAs	National Health Priority Areas
NSI	National Cancer Institute
NSWOG	NSW Oncology Group
OCPs	optimal care pathways
PBAC	Pharmaceutical Benefits Advisory Committee
PBS	Pharmaceutical Benefits Scheme
PCA	Pancreatic Cancer Alliance
PdCCRS	Priority-driven Collaborative Cancer Research Scheme
QALYs	quality-adjusted life years
QBI	Queensland Brain Institute
QIMR Berghofer	QIMR Berghofer Medical Research Institute
RACGP	Royal Australian College of General Practitioners
RCA	Rare Cancers Australia
Roche	Roche Products Pty Limited
RSP	research support package

STIs	sexually transmitted infections
TBTC	The Brain Tumour Charity
TCR	Targeted Call for Research
TGA	Therapeutic Goods Administration
the Act	United States *Recalcitrant Cancer Research Act of 2012*
the Committee	Select Committee into Funding for Research into Cancers with Low Survival Rates
The Isabella and Marcus Fund	The Isabella and Marcus Paediatric Brainstem Tumour Fund
the Mission	Australian Brain Cancer Mission
the Plan	National Cancer Work Plan
the Strategy	the *Australian Medical Research and Innovation Strategy 2016–2021*
UK	United Kingdom
UQ	University of Queensland
US	United States
VCCC	Victorian Comprehensive Cancer Centre
VCTL	Victorian Cancer Trials Link
Walter and Eliza Hall Institute	The Walter and Eliza Hall Institute of Medical Research

Recommendations

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.

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.

Recommendation 3

3.85 The committee recommends that the Australian government improves AustralianClinicalTrials.gov.au so it is more accessible and user-friendly.

Recommendation 4

3.91 The committee recommends that state and territory governments consider:

- **allowing low survival rate cancer patients participating in clinical trials to access patient travel subsidy schemes; and**
- **agreeing on consistent subsidy rates based on the distance and method of travel, and the average cost of accommodation in the city in which the patient is participating in the trial.**

Recommendation 5

3.93 The committee recommends that Australian governments improve access to international clinical trials for people with low survival rate cancers, including by:

- **exploring ways to reduce the financial barriers to accessing international trials to the extent possible; and**
- **further developing the existing capacity for international collaboration on trials.**

Recommendation 6

3.129 The committee recommends that Australian governments, as a priority, further streamline ethics and governance approval processes for clinical trials, particularly where those processes differ between states and territories, and public and private research institutions.

Recommendation 7

3.132 The committee recommends that the National Health and Medical Research Council develops a standard template and associated guidelines, including timeframes, for ethics and other governance approvals for consideration and possible adoption by each state and territory.

Recommendation 8

4.33 The committee recommends that, through the Council of Australian Governments Health Council, the Australian government leads a process to ensure that arrangements for transitioning children and young people from paediatric to adult oncology services occurs in a consistent and co-ordinated way that preserves continuity and quality of care in the best interests of each individual patient.

Recommendation 9

5.20 The committee recommends that the Australian government undertakes communication activities targeted at the public with the objective of reducing the amount of time taken to detect and diagnose low survival rate cancers.

Recommendation 10

5.25 The committee recommends that the Australian government works in collaboration with the Royal Australian College of General Practitioners and the Australian Medical Association to improve awareness of low survival rate cancers amongst general practitioners, including through continuing professional development.

Recommendation 11

5.59 The committee recommends that the Australian government, in collaboration with state and territory governments:

- **considers expanding the Australian Cancer Database to capture all cancers, including benign tumours of the brain and other parts of the central nervous system;**
- **in so doing, consults with medical researchers to identify what clinical and lifestyle data might be included in order to benefit oncology research; and**
- **addresses current barriers to data collection and considers ways in which data collection can be improved across Australia, in both public and private health settings.**

Recommendation 12

5.62 The committee recommends that the Australian government gives serious consideration to implementing a national network medical and population biobank that includes tumour samples and relevant clinical and lifestyle data associated with each tumour sample.

Recommendation 13

5.98 The committee recommends that the Australian government ensures ongoing funding for genomic research into low survival rate cancers.

Recommendation 14

5.99 The committee recommends that the Australian government implements any recommendation from the Medical Services Advisory Committee to list genetic tests for low survival rate cancer patients on the Medicare Benefits Schedule so that these tests are routinely available to these patients and reimbursed.

Recommendation 15

5.102 The committee recommends that the Therapeutic Goods Administration, if necessary following the medicines and medical devices review, and the Pharmaceutical Benefits Advisory Committee:

- **(re-)examine their assessment processes and the appropriateness of those processes for innovative treatments for low survival rate (LSR) cancers, such as immunotherapies; and**
- **pending that examination, consider adopting more flexible and innovative approaches to approving innovative treatments for LSR cancers and assessing them for listing on the Pharmaceutical Benefits Scheme.**

Recommendation 16

5.126 The committee recommends that the Australian government ensures funding is available to researchers investigating whether existing drugs may be suitable for treating low survival rate cancers.

Recommendation 17

5.128 The committee recommends that the Australian government works with industry to consider a mechanism to repurpose drugs.

Recommendation 18

5.131 The committee recommends that the Australian government considers a mechanism to permit access to and properly supervise use of off-label drugs for low survival rate cancer patients without further treatment options, on compassionate grounds.

Recommendation 19

5.133 The committee recommends that the Therapeutic Goods Administration and Pharmaceutical Benefits Advisory Committee examine the appropriateness of their approval and assessment processes for existing drugs repurposed for use in low survival rate cancers.

Recommendation 20

5.136 The committee recommends that the Australian government considers whether the Medical Services Advisory Committee and Pharmaceutical Benefits Advisory Committee processes can be streamlined where a diagnostic test and treatment for a low survival rate cancer are co-dependent.

Recommendation 21

5.176 The committee recommends that the Australian government, in conjunction with its state and territory counterparts, works to improve access to specialist cancer care co-ordinators or nurses for low survival rate cancer patients in every state and territory.

Recommendation 22

5.179 The committee recommends that the Australian government asks the Medical Services Advisory Committee to review the criteria for reimbursement of ongoing diagnostic testing for low survival rate cancer patients.

Recommendation 23

5.181 The committee recommends that the Australian government further simplifies and streamlines the application process for low survival rate cancer patients and their carers when seeking to access the Disability Support Pension, or carer allowance or payment.

Recommendation 24

5.223 The committee recommends that the federal, state and territory governments develop and implement a comprehensive Australia-wide strategy to increase 5-year survival rates for low survival rate cancers to above 50 per cent by 2027:

- **taking into account the recommendations in this report;**
- **consulting with researchers, clinicians, patients and patient groups;**
- **considering the roles of research, early diagnosis and access to medicines; and**
- **assessing the applicability of international approaches, such as the *Recalcitrant Cancer Research Act of 2012 (US)*, to the Australian context.**

Recommendation 25

5.225 The committee recommends that annual progress reports on the development and implementation of an Australian strategy to improve survival rates for low survival rate cancers are provided to the Council of Australian Governments Health Council and made publicly available.

Chapter 1
Introduction

Referral and conduct of the inquiry

1.1 On 29 November 2016, the Senate established the Select Committee into Funding for Research into Cancers with Low Survival Rates (the committee) to inquire and report by 28 November 2017 on:[1]

> a. the current National Health and Medical Research Council [(NHMRC)] funding model, which favours funding for types of cancer that attract more non-government funding, and the need to ensure the funding model enables the provision of funding research into brain cancers and other low survival rate cancers;
>
> b. the obstacles to running clinical trials for brain cancers and other cancers with relatively lower rates of incidence, with regard to:
>
> > i. funding models that could better support much-needed clinical trials, and
> >
> > ii. funding support for campaigns designed to raise awareness of the need for further research, including clinical trials;
>
> c. the low survival rate for brain cancers, lack of significant improvement in survival rates, and strategies that could be implemented to improve survival rates and;
>
> d. other relevant matters.[2]

1.2 The committee received and published 326 submissions, listed at Appendix 1.

1.3 The committee took evidence from 117 witnesses over seven days of public hearings in:

- Sydney on 18 and 19 May 2017;
- Brisbane on 6 June 2017;
- Melbourne on 7 June 2017;
- Canberra on 8 June 2017;
- Melbourne on 4 August 2017; and
- Canberra on 29 August 2017.

1.4 The witnesses who appeared at these hearings are listed at Appendix 2.

1.5 The committee also received a number of additional documents, and answers to questions on notice also listed at Appendix 1.

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1 *Journals of the Senate*, No. 21, 29 November 2016, pp 662–663.

2 *Journals of the Senate*, No. 21, 29 November 2016, p. 662.

Structure and scope of this report

1.6 This report comprises six chapters:

- chapter 1 outlines the conduct of the inquiry, and the definitions for low survival rate (LSR) cancers, distinct from 'rare cancers';
- chapter 2 examines in particular funding for LSR cancers;
- chapter 3 discusses clinical trials for LSR cancers;
- chapter 4 discusses paediatric and youth cancers; and
- chapter 5 considers ways in which survival rates can be increased for LSR cancers.

Definitions

1.7 In responding to the terms of reference for the inquiry, submitters and witnesses to the committee referred both to LSR cancers and rare cancers.

1.8 For example, the Low Survival Cancers Alliance, comprising 11 organisations including the Cancer Council Victoria and the Leukaemia Foundation, defined LSR cancers as:

> …those with five year survival less than or equivalent to 30% and include mesothelioma, pancreas, liver, lung, oesophagus, gallbladder, brain, adult acute myeloid leukaemia, stomach, some neuroendocrine cancers (NETs) and cancer of unknown primary.[3]

1.9 This definition was also put forth by other submitters, including Cancer Voices Australia[4] and the Australasian Gastro-Intestinal Trials Group.[5]

1.10 By contrast, Cancer Australia stated that '[a]s there is no standard definition' of LSR cancers, and focussed in its submission 'on eight cancer types which all have a <50% five-year relative survival rate'.[6] Those cancers identified were ovarian, stomach, acute myeloid leukaemia, brain, oesophageal, lung, pancreatic and mesothelioma cancers.[7]

1.11 The reference to less than 50 per cent survival rate was also identified by the Australian Institute of Health and Welfare (AIHW).[8] In its submission, AIHW identified that in 2009–2013, the following cancers fell into that category:

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3 Low Survival Cancers Alliance, *Submission 90* p. 1. The other members of the alliance are the Asbestos Council of Victoria, AsbestosWise, the Bernie Banton Foundation, Brain Tumour Alliance Australia, the Isabella & Marcus Fund, the Lung Foundation Australia, Pancare, Robert Connor Dawes and the Unicorn Foundation.

4 Cancer Voices Australia, *Submission 61*, p. 1.

5 Australasian Gastro-Intestinal Trials Group, *Submission 85,* p. 1.

6 Cancer Australia, *Submission 129*, p. 1.

7 Cancer Australia, *Submission 129*, p. 1.

8 Australian Institute of Health and Welfare (AIHW), *Submission 83*, p. 2.

mesothelioma, pancreas, unknown primary site, lung, liver, gallbladder, oesophagus, brain, acute myeloid leukaemia and stomach.[9]

1.12 The committee has accepted this latter definition of LSR cancers for the purpose of this report, that is cancers with less than a 50 per cent survival rate.

1.13 The committee also notes that Rare Cancers Australia identified the distinction between 'less common' and 'rare cancers':

> **'Less common'** cancers as those with an incidence of between 6 and 12 (inclusive) per 100,000 Australians per annum.
>
> **'Rare cancers'** are defined as those with an incidence of less than 6 per 100,000 Australians per annum – a total of 186 cancer types have been defined as rare.[10]

1.14 The committee acknowledges the distinction between LSR, less common and rare cancers. However, the committee will refer only to LSR cancers unless otherwise specified.

A note on the NHMRC funding model

1.15 Since the inquiry was first announced, the NHMRC has made certain changes to its funding structure. Most of the submissions and some of the evidence received at the committee's public hearings were provided in the context of the former NHMRC funding structure.

1.16 Unless specified, the committee believes that the evidence it has included in its report is also reflective of the current funding structure.

1.17 The changes that were announced on 25 May 2017,[11] and the effect of these changes, is discussed in more detail in chapter 2.

Recent funding announcements

Funding for rare cancers

1.18 On 24 August 2017, the Australian government announced that Australian children would have access to AIM BRAIN, a four year study with the aim of transforming the brain tumour classification, treatment and survival of children with brain cancer.[12] Further information about this announcement appears in chapter 3.

1.19 The government also announced $13 million of funding for competitive research grants through the Medical Research Future Fund (MRFF), which is targeted

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9 AIHW, *Submission 83*, p. 2.

10 Rare Cancers Australia, *Submission 50*, p. 1.

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

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

towards rare cancers and rare diseases.[13] Further information about this announcement appears in chapter 2.

The Australian Brain Cancer Mission

1.20 On 29 October 2017, the Australian government announced the Australian Brain Cancer Mission, a $100 million fund to defeat brain cancer.[14] Further information about this fund appears at chapter 5.

Acknowledgements

1.21 The committee thanks individuals and organisations that contributed to the inquiry, and takes this opportunity to express its gratitude to those individuals who took the time to share their personal stories with the committee.

1.22 The committee appreciates that for some, sharing their personal experiences was difficult and upsetting. The committee was deeply moved by these stories and the inquiry has benefitted from their being shared.

Notes on references

1.23 References to the *Committee Hansard* may be references to the proof transcript. Page numbers may differ between proof and official transcripts.

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

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

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.

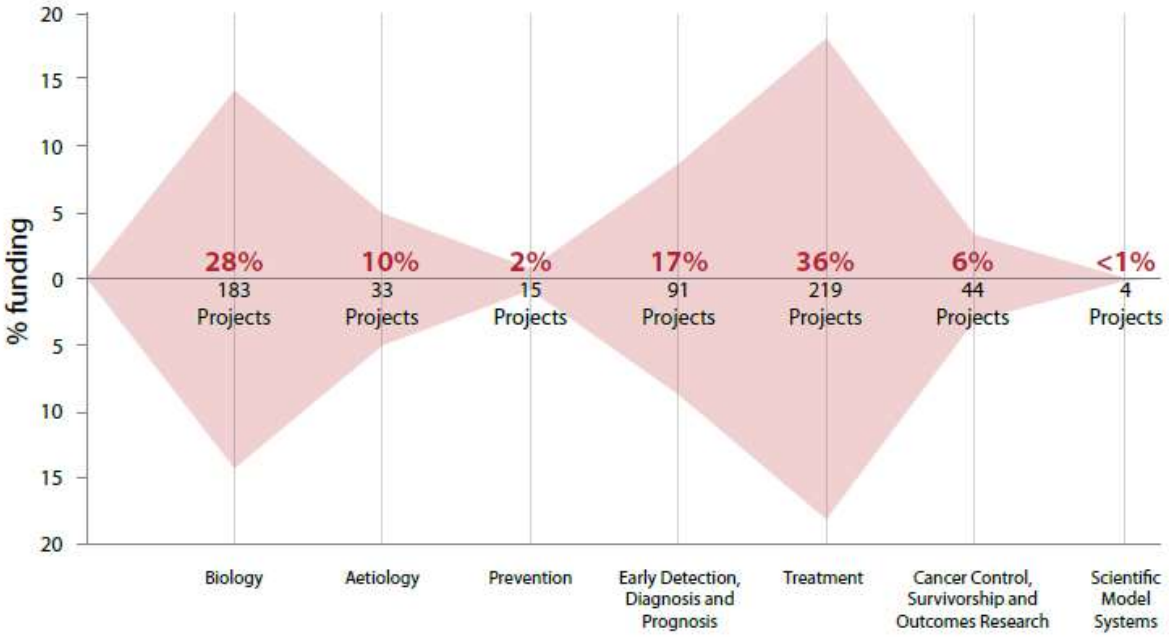
3 Cancer Australia, *Cancer Research in Australia 2016 to 2018 - Opportunities for strategic research investment – Highlights*, 2016.

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.

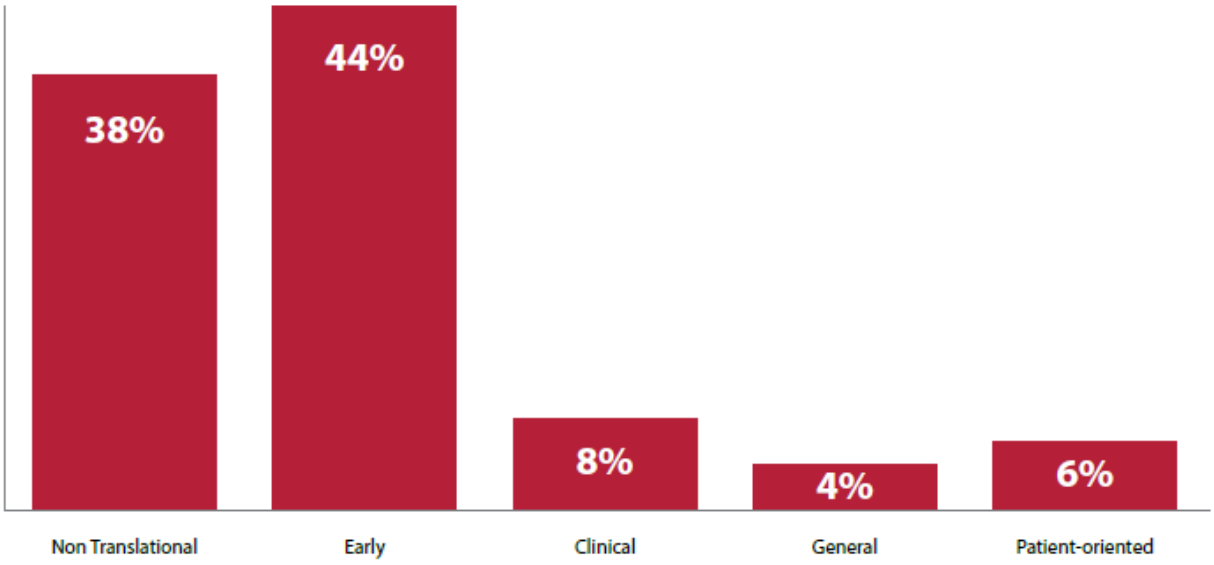
7 Cancer Australia, *Cancer Research in Australia 2016 to 2018 - Opportunities for strategic research investment – Highlights*, 2016, p. 2.

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.

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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]

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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.

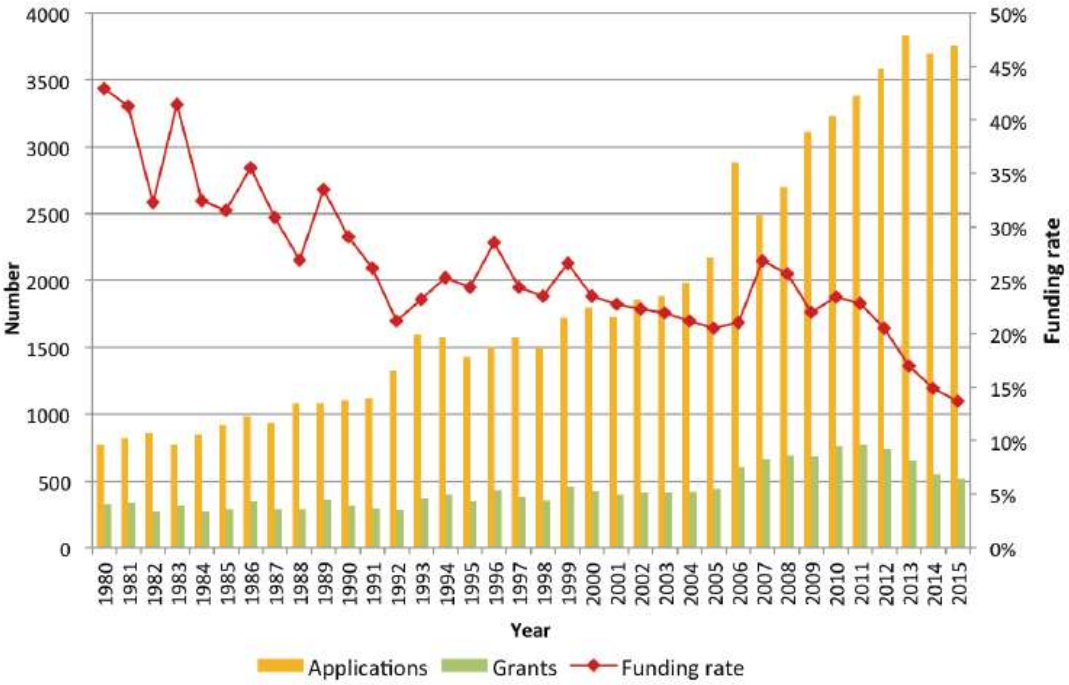
49 Research Australia, *Submission 122*, pp 7–8.

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

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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.

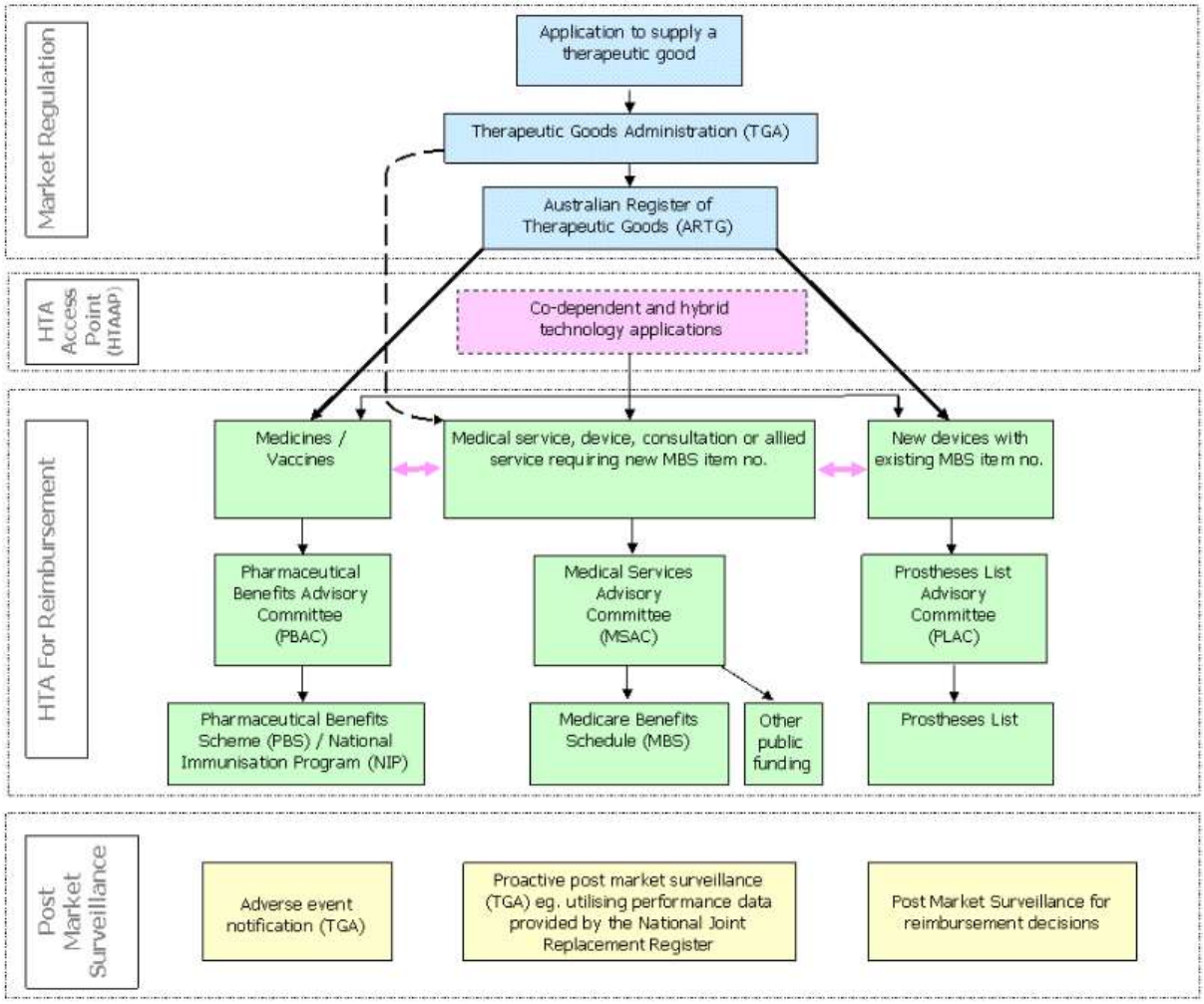
148 For the principles and methodologies used by the PBAC, see: DoH, *Guidelines for preparing a submission to the Pharmaceutical Benefits Advisory Committee*, September 2016, p. 4.

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).

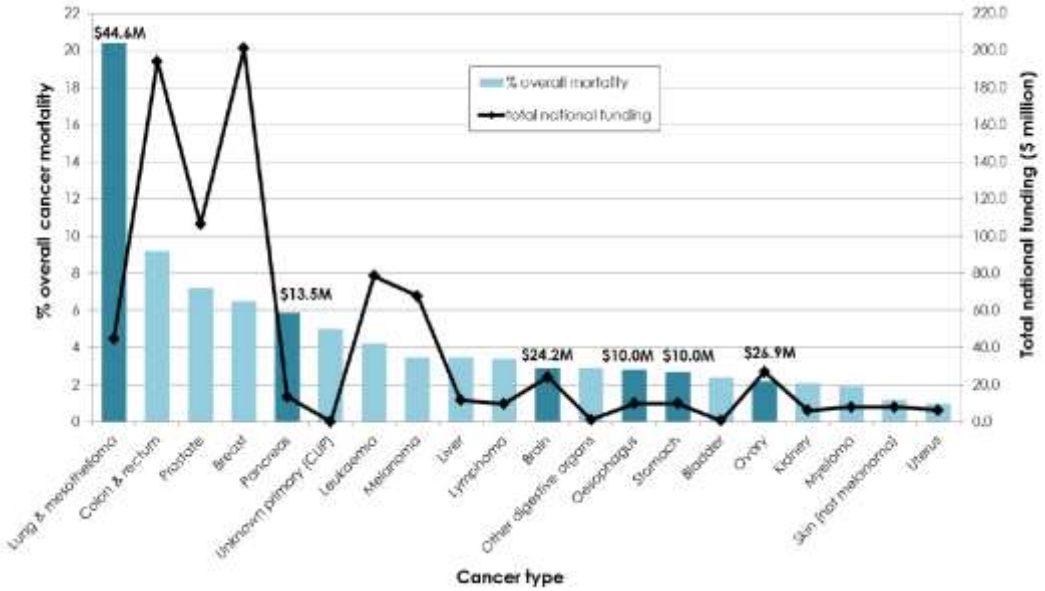
177 Dr Nicola Waddell, Group Leader, Medical Genomics Group, QIMR Berghofer, *Committee Hansard*, 6 June 2017, p. 45.

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]

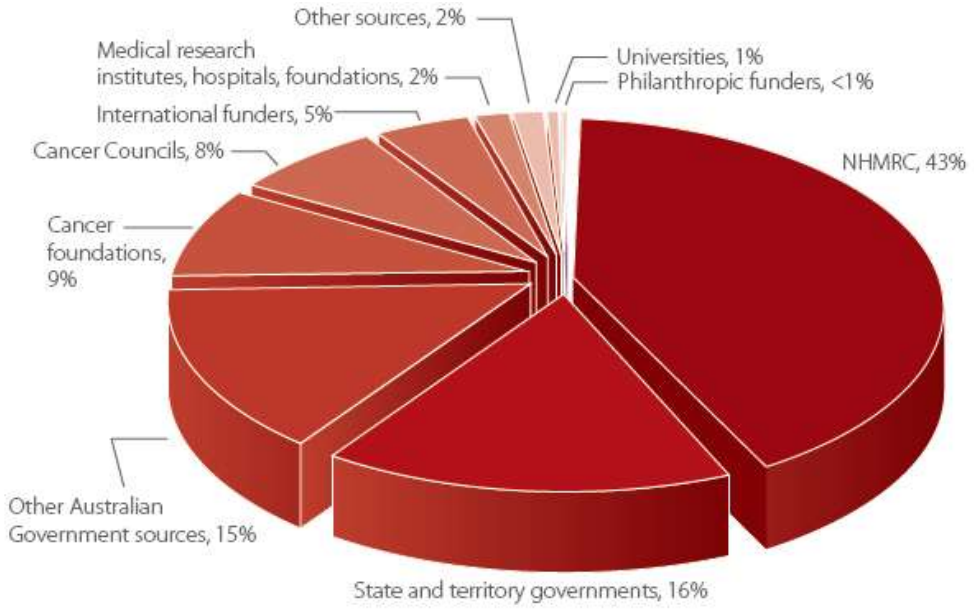
181 Cancer Australia, *Submission 129*, p. 5. This excludes data for acute myeloid leukaemia, which was not available.

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.

Chapter 3

Clinical trials for people with low survival rate cancers

3.1 This chapter examines two distinct issues with respect to clinical trials for people with low survival rate (LSR) cancers: barriers to accessing trials and jurisdictional issues for trials.

3.2 The Garvan Institute of Medical Research/The Kinghorn Cancer Centre/The Garvan Research Foundation (Garvan Institute) outlined in its submission the existing ways in which patients without any standard treatment options can access clinical trials, and noted that '[t]he first step in improving the outcomes for rare and high-mortality cancers is to engage patients in the research enterprise. Without data, nothing can improve'.[1] The treatment access options are as follows:

- phase 1 clinical trials – as these are primarily focused on defining the toxicity profile of a new treatment, it takes a long time to get sufficient numbers of participants and they are costly and intensive, limiting the number of phase 1 studies that a single institution can open at one time.
- phase 2 or 3 clinical trials, however, cost limits the number of phase 2 studies that can be run simultaneously at any one institution.
- compassionate access to new drugs and off-label treatment. This is common practice in Australia, and while it may produce anecdotal insight into novel therapeutic possibilities, these results are idiosyncratic, ad hoc, unsupervised and unregulated, and mostly go unreported, thus failing to contribute to the body of knowledge. Most importantly, ineffective treatment is likely to be underreported.[2]

3.3 The Garvan Institute further noted that the 'two key barriers to improved outcomes for less common cancers' are lack of access to clinical trials, and lack of access to the best available treatments, which are '[i]nextricably linked', because:

> As governments use information gained from trials when deciding if they will fund a new drug, it is critical that patients with less common cancers have access to clinical trials, and that government, academics, clinicians and the pharmaceutical industry work together to develop trials for these cancers, as well as the more common cancers. Currently, there is a real disconnect between the identification of a new treatment by researchers and, where relevant, access to these treatment options.[3]

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1 Garvan Institute of Medical Research/ The Kinghorn Cancer Centre/ The Garvan Research Foundation (Garvan Institute), *Submission 34*, p. 5. The Garvan Institute defines rare and less common cancers as those 'affecting up to 12 in 100,000 people', which 'account for 23.7% of cancers diagnosed, and 38.5% of cancer deaths': p. 2.

2 Garvan Institute, *Submission 34*, p. 5.

3 Garvan Institute, *Submission 34*, p. 4.

3.4 The relationship between clinical trials and the Therapeutic Goods Administration (TGA), the Pharmaceutical Benefits Advisory Committee (PBAC) and the Pharmaceutical Benefits Scheme (PBS), as well as philanthropic and pharmaceutical funding for clinical trials, were examined more generally in chapter 2.

Barriers to accessing trials

3.5 There are a number of barriers to accessing trials, including the absence of trials for LSR cancers, identifying the availability of trials, meeting the trial criteria and having the physical and financial means to participate in a domestic or international trial.

3.6 The Walter and Eliza Hall Institute of Medical Research (Walter and Eliza Hall Institute) identified the following 'recurring themes' with respect to the challenges to establishing clinical trials in Australia:

> i) access is limited for patients with rare cancers, as trials will not be available in all major treatment centres;
>
> ii) access for patients in rural Australia is difficult when the trial requires frequent attendance at a capital city centre;
>
> iii) the time taken to establish a trial is disproportionately long compared to the survival time of patients with low survival cancer; and
>
> iv) pharmaceutical companies are risk adverse when it comes to initiating adequately sized trials in cancers with low incidence.[4]

3.7 Mr Tim Eliot identified several barriers to his participation in clinical trials which caused him to accept standard of care treatments[5] for his glioblastoma:

> …admin did not provide details of the trial; existing treatment timing meant the trial start date was missed by a week; my tumour was in the wrong location; the trial was already full; the trials were not being run in Western Australia; etc, etc.[6]

4 The Walter and Eliza Hall Institute of Medical Research (Walter and Eliza Hall Institute), *Submission 126*, p. 5.

5 Namely, '[t]reatment that is accepted by medical experts as a proper treatment for a certain type of disease and that is widely used by healthcare professionals. Also called best practice, standard medical care, and standard therapy' – see, National Cancer Institute, *NCI Dictionary of Cancer Terms*, https://www.cancer.gov/publications/dictionaries/cancer-terms?cdrid=346525 (accessed 3 November 2017).

6 Mr Tim Eliot, *Submission 43*, p. 2.

3.8 Mr Eliot opined that:

> …these symptoms show the current funding model is based on standard clinical research practices with a limited number of patients able to be involved, and little, if any, data sharing between trials.[7]

3.9 Mr Eliot argued that '[t]his approach is simply not working'[8] and although he acknowledged that '[t]here are valid reasons for clinical standards to be set high, particularly in researching new treatments',[9] he submitted that 'standard, slow, phased clinical trials are not the only way forward' and discussed the GBM AGILE model as an alternative.[10] The Cure Brain Cancer Foundation (CBCF) noted that this particular trial had an 'innovative trial design' and 'an adaptive trial platform, which has great potential to reduce timeline[s] through seamless transition from Phase 2 to Phase 3 within the trial'.[11] Access to this trial is further discussed in chapter 5.

3.10 The following sections examine some barriers to accessing trials that were repeatedly cited during the course of the committee's inquiry.

"Dr Google"

3.11 In addition to people independently searching the internet for information about inexplicable symptoms[12] or about a disease following diagnosis,[13] the committee heard that people resort to "Dr Google" to find out information about access to trials. For example, Ms Marilyn Nelson, who has lung cancer, described how she conducted her own research on clinical trials in order to find 'some hope':

> We are looking for news about trials and new drugs that are coming along. There is not that much information about it here in Australia, so we look to Google and we look to proper websites over there to try to find—just some hope, you know? That is what we are looking for, that there might be something.[14]

3.12 Professor Mark Hertzberg of the Australasian Leukaemia and Lymphoma Group (ALLG) considered that it is now easier, with the internet, to access information about clinical trials, noting that patients:

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7 Mr Eliot, *Submission 43*, pp 2–3.

8 Mr Eliot, *Submission 43*, p. 3.

9 Mr Eliot, *Submission 43*, p. 3.

10 Mr Eliot, *Submission 43*, p. 3.

11 Cure Brain Cancer Foundation, *Submission 139*, p. 8.

12 Mrs Raechel Burgett, *Submission 53*, p. 1.

13 See, for example, Mrs Sandra Woods, *Submission 7*, p. 3; Mrs Michelle Patterson, *Submission 13*, p. 1; Ms Sherrin Bell, *Submission 276*, p. 3.

14 Ms Marilyn Nelson, *Committee Hansard*, 6 June 2017, p. 7.

> …come with wads of paper, particularly the relatives of the patient and the children of the patient. The clinicians also have better access [to information about trials] than ever before.[15]

3.13 Indeed, Professor David Walker opined that the approach of searching the internet for treatments occurs '[a]ll the time', as:

> …a patient with a brain cancer will be told they need an operation, they will be told the diagnosis and 'This is what is going to happen to you'. There is very little information given to them up-front and there is certainly no information in almost all circumstances about available trials and what that might have to offer.[16]

3.14 Ms Julie Marker of Cancer Voices Australia also suggested that clinicians may be unfamiliar with LSR cancers and associated trials, which in turn can raise a whole host of problems:

> Often clinicians are not so familiar with these rare cancer types and the trials. So there may well be opportunities for treatments that people are just not aware of both from the clinicians and from the consumers side of it to find the best treatments, or even the clinicians who are in any way familiar with treating these conditions. Often that means travelling to other locations. Again, you have to be wealthy enough to afford to do that because that is not supported. There is the potential for duplication if there is not some register of even the preliminary pilot studies.[17]

3.15 This issue about the lack of information available to patients was also reflected in the evidence from Mr Evan Shonk and Mrs Suzanne Turpie:

> **CHAIR:** One of the other things that a number of you have mentioned was clinical trials. I am interested in what sort of information you were given about clinical trials and how much you had to go away and research for yourselves.
>
> **Mr Shonk:** There is virtually none available. They pretty much do not exist.
>
> **CHAIR:** Someone mentioned to me that there are not even any brochures available about brain cancer.
>
> **Mrs Turpie:** Yes. We did not encounter any brochures. We were just sat in a room and told, 'This is a clinical trial that kids with medulloblastoma are on.' To be honest, there was nothing given to us, it was scary and I felt like my son was being used for research himself while he was still living.[18]

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15 Professor Mark Hertzberg, Member and Director, Australasian Leukaemia and Lymphoma Group (ALLG), *Committee Hansard*, 7 June 2017, p. 31.

16 Professor David Walker, *Committee Hansard*, 6 June 2017, p. 51.

17 Ms Julie Marker, Executive Teams, Cancer Voices Australia and Cancer Voices South Australia, *Committee Hansard*, 18 May 2017, p. 16.

18 Mr Evan Shonk and Mrs Suzanne Turpie, *Committee Hansard*, 18 May 2017, p. 8.

3.16 Similarly, in her evidence to the committee, Ms Jill Emberson, who has ovarian cancer, expressed her surprise that 'there is not more clear information available about running trials', but also identified why this may be the case:

> …I understand that all the people running the trials are so strapped in even getting their trials up and running and that the administrative support, as I understand, is also a real barrier to people running the trials inside the hospitals and the labs. And that that would be stopping trials getting up and running, I find, gobsmacking.[19]

3.17 Mr William Williams, whose wife passed away from a GBM grade 4, also spoke of his experience of finding out information about trials:

> There is a website that I did look at, which did not really lead me anywhere in particular to the possibility of a trial. So it was in fact drawing on the experience of Denis and other people I knew in the brain tumour area, and just saying, 'Who do you think might be running a trial?' Denis said, 'Well, you can call so and so in Melbourne', and I did. After announcements of other initiatives in cancer research in this country, I called and just said who I was. But there was no coordination or leading me in any way that showed me a direction where there could be a trial. So you just cold-call and say who you are and say, 'Can you help?' because it was not available.[20]

3.18 As Mr Todd Harper of the Cancer Council Victoria (CCV) observed, the motivation of cancer patients to seek out the clinical trials available to them 'speaks to the value of having information that is consumer friendly and is able to guide them towards these types of activities'.[21]

AustralianClinicalTrials.gov.au

3.19 The committee was informed that information about trials is available on the AustralianClinicalTrials.gov.au website, developed by Cancer Australia in partnership with the Australian New Zealand Clinical Trials Registry, the University of Sydney and Cancer Voices.[22]

3.20 Dr Alison Butt of Cancer Australia provided the following information about the website:

> …the Cancer Australia website supports the only national cancer clinical trials website which gives consumers access to current clinical trials in Australia and to Australian arms of international trials. A particular focus of the website is that it's consumer friendly. So there are consumer lay descriptions of the trials, which obviously help when patients are looking for appropriate trials. There are simple search functions which enable them

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19 Ms Jill Emberson, Patient and Consumer Advocate, Ovarian Cancer Australia, *Committee Hansard*, 4 August 2017, p. 4.

20 Mr William Williams, *Committee Hansard*, 8 June 2017, p. 5.

21 Mr Todd Andrew Harper, Chief Executive Officer, Cancer Council Victoria (CCV), *Committee Hansard*, 4 August 2017, p. 34.

22 Cancer Australia, *Submission 129*, p. 7.

> to navigate through the site and find trials that are eligible. In addition, there's also specific information about the eligibility of the trials and the implications of the trial participation. So the focus of the website is really aimed at trying to encourage participation by making it a very user-friendly experience.
>
> As you alluded to, the data for the Australian cancer clinical trials website is sourced from the Australian New Zealand Clinical Trials Registry, the ANZCTR, but also ClinicalTrials.gov, which is the US clinical trials website. It is dependent on the clinical trials being registered, and the responsibility rests with the investigator and sponsor of the clinical trials, so that is potentially a challenge. It is their responsibility to update and provide information on that website.[23]

3.21 Although this description indicates that the website contains a wealth of information about clinical trials, the committee heard that people living with cancer are not accessing this information due to the difficulty they experience navigating the website.[24]

3.22 Mr Greg Mullins of Research Australia proffered why this may be the case:

> I think it is extremely difficult for individual patients to know what clinical trials might be suited to them. In nearly all cases they are going to be relying on their treating doctor to be able to assist them to understand whether they are eligible or not. There are searches that can be done and, if someone perhaps gets really lucky and really knows what they are doing, they might be able to find that information, but most people are going to be relying on their doctors to assist them with that.
>
> We have undertaken public polling in the past, where we have asked people about clinical trials: are they aware of them and what they are? Typically, what they are telling us is: 'I rely on my doctor.' That is very much where it is at. I know the last speakers were talking about the difficulty of understanding even the range of clinical trials happening within Victoria. On a global scale, that is enormous, and it is not the patients who are in a position to do that. It really is a matter of ensuring that our researchers and our organisations are connected globally and understand what is happening.[25]

3.23 Indeed, Professor R John Simes considered that doctors are the 'main people' who view the AustralianClinicalTrials.gov.au website, but noted that the website, which contains 'a lay description so that the information is in less threatening terms', is

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23 Dr Alison Butt, Senior Scientific Officer, Cancer Australia, *Committee Hansard*, 29 August 2017, p. 25.

24 See, for example, Ms Christine Christensen, Chair, Cancer Voices South Australia, and Executive Member, Cancer Voices Australia, *Committee Hansard*, 18 May 2017, p. 18.

25 Mr Greg Mullins, Head of Policy, Research Australia, *Committee Hansard*, 7 June 2017, p. 45.

also accessible to members of the community.[26] Despite this, Professor Simes considered that improvements could be made to the website, as:

> …to work out whether a particular trial is suitable for a particular patient still requires a discussion with their doctor et cetera…while one thing is to be able to find out what trials are available, the other thing is—if the trial is not available at your site, in your city or at your hospital—how can you get access to other places. They are really important issues.[27]

3.24 This was reflected in Mrs Madeline Bishop's submission, where she asserted that, when looking at the website:

> …one needs to know exactly what one is looking for to be able to locate and be included in a trial. When looking for non-government or partially funded trials, one must seek information from the individual groups and their current trials. This haphazard method is not good enough for the individual whose health and wellbeing is already compromised by their cancer.[28]

3.25 Indeed, Ms Susan Pitt also informed the committee that some trials, such as physician-led trials, may not be listed on the website.[29]

3.26 In its submission, the National Health and Medical Research Council (NHMRC) referred to survey results which 'demonstrate that the lack of awareness of relevant trials is a barrier, not just to increased participation, but also to increased cross-referral of patients by general practitioners or clinicians'.[30]

3.27 In response to this, the NHMRC has been 'working to improve recruitment into and awareness of clinical trials' in the following ways:

> a) enhancing the functionality of the AustralianClinicalTrials.gov.au website to bring together resources for consumers, participants, researchers and proponents of clinical trials, and as a tool to encourage patient recruitment, and
>
> b) developing a national marketing campaign to improve awareness of the website and an understanding of the role and value of clinical trials. Funding for the campaign has been provided by the Department of Industry, Innovation and Science.

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26 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. 47.

27 Professor Simes, Cooperative Trials Group for Neuro-Oncology and University of Sydney, *Committee Hansard*, 18 May 2017, pp 47–48.

28 Mrs Madeline Bishop, *Submission 35*, p. 2.

29 Ms Susan Pitt, *Committee Hansard*, 8 June 2017, p. 11.

30 National Health and Medical Research Council (NHMRC), *Submission 87*, p. 5.

3.28 The NHMRC noted that '[i]mprovements in cross-referral rates of GPs and clinicians have also been observed through the use of a Mobile Applications ('Apps') - ClinTrials refer'.[31]

3.29 However, in response to a question about the accessibility of the website, Adjunct Associate Professor Christine Giles of Cancer Australia conceded:

> We can certainly look at different ways of directing people to the website—through social media and some of our existing mechanisms. The consumer organisations, we would anticipate, would do that as well. But, given the comments that you're making, we would certainly be able to have a look at that.[32]

3.30 Mr Harper spoke to the committee about the CCV's clinical trials website, Victorian Cancer Trials Link (VCTL), informing the committee that, following a redevelopment, the CCV had successfully made the website 'more user-friendly and searchable for individuals' and as a result, 'there has been quite a lot of interest right across Australia' and internationally.[33] Mr Harper elaborated on the redevelopment process:

> The recent website redevelopment was done with patients. We wanted to make sure that the final product was one that was very user friendly. Since the redevelopment of the website we saw in May this year the website attracted 3,130 visits from users, which was a 30 per cent increase from prior to the introduction of the new website. At least on those initial numbers we are very confident that it has responded to the need of cancer patients.[34]

3.31 Mr Harper suggested that the CCV clinical trials could be 'made available more broadly', noting that:

> I am sure that my Cancer Council colleagues, for whom clinical trials is a priority, would be very happy to work on expanding what was essentially a prototype developed in Victoria and making that available nationally. Obviously, having a site that is already established and has demonstrated feasibility may offer some advantages.[35]

3.32 However, Mr Harper identified that some issues would need to be addressed, including:

> …encouraging clinical trial sites to contribute data. That is done under a funding model in Victoria, as I said. Currently it is about $200,000 in Victoria. Ideally, we would like to increase that in Victoria to make that available or provide a greater incentive for organisations to submit their

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31 NHMRC, *Submission 87*, p. 5 (citations omitted).

32 Adjunct Associate Professor Christine Giles, Executive Director, Cancer Australia, *Committee Hansard*, 29 August 2017, p. 25.

33 Mr Harper, CCV, *Committee Hansard*, 4 August 2017, p. 33.

34 Mr Harper, CCV, *Committee Hansard*, 4 August 2017, p. 34.

35 Mr Harper, CCV, *Committee Hansard*, 4 August 2017, p. 33.

> trial's information. I do not see any reason why that could not be looked at as a prototype that could be rolled out across Australia. My guess is that, if that was done, funding would be between $1 million and $2 million, probably closer to two, to enable incentives for trial sites to submit their data and also to upkeep the website and promote that website.[36]

3.33 Mr Harper outlined the benefits of this approach:

> The two principal benefits of that that, I see, are: firstly, to provide access in a form that has been demonstrated to work well with consumers; and secondly, to enable trial sites to use that to recruit patients to their clinical trials. I think that that would be quite a substantial benefit as well. I should also note that the Ian Potter Foundation was very generous in providing us funding to enable the website to be recently redeveloped.[37]

Eligibility for trials

3.34 Many people who have LSR cancers may be ineligible for trials because of their current state of health, prior treatment, or their age.

3.35 For example, Ms Linda Ferguson, who lost her wife to brain cancer, informed the committee that:

> We asked our various specialists in Canberra and in Gosford if there were any trials that were suitable. We were told that there were not. We did research online to see if we could come up with anything, but we found that, once you make particular treatment choices, you are given a particular drug or the tumour recurs, suddenly anything that might have been eligible you are no longer eligible for, because you have already had another drug. So the doors close very quickly once you have made treatment options. With time being such a pressure, you make those treatment decisions as quickly as you can.[38]

3.36 Mrs Raechel Burgett, who has a grade-3 oligoastrocytoma, stated that to access certain trials, she would need to be on her 'deathbed':

> I looked and I applied but it is all for grade 4 astrocytomas because that is the worst and the deadliest. They are opening all the trials for them, and even at that stage it is not until you are terminal that they really let you in. I am someone who is still relatively early in their diagnosis and who has a few years up their sleeve, and so they will not me let in until I am on my deathbed.[39]

3.37 Mrs Tracy Taylor also described how her son, who has brain cancer, could not access trials for various reasons, including his prior treatment and age:

> My son has already had the gold standard of treatment and radiation a couple of times, so that in itself makes him not applicable for trials. His age

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36 Mr Harper, CCV, *Committee Hansard*, 4 August 2017, p. 33.

37 Mr Harper, CCV, *Committee Hansard*, 4 August 2017, p. 33.

38 Ms Linda Ferguson, *Committee Hansard*, 18 May 2017, p. 9.

39 Mrs Burgett, *Committee Hansard*, 6 June 2017, p. 6.

> as well makes him not eligible for trials. If they know from the start and they have other treatments, as they are calling them, or ones that are yet to be made and they are yet to trial that they can maybe go on this path of this new thing, as opposed to just doing the gold standard of treatment, which then makes them not eligible to do other trials.[40]

3.38 The timeframe within which a person can be eligible to begin a trial can also be quite tenuous. For example, Ms Simone Leyden of the Unicorn Foundation recounted a story of a patient who managed to join to a trial after her oncologist initially informed this patient that no trials were available. Ms Leyden noted that '[i]f she had literally waited another 24 hours, she would not have been eligible for that trial'.[41]

3.39 In addition to the eligibility criteria, Ms Nelson informed the committee that '[t]here is a strict protocol' when you are in a trial, explaining that:

> I cannot have had this and I cannot have had that to get into the trial. Then, while I am on the trial, I cannot use any other therapy. If I do, my doctor would have to agree to it. The only reason they would stop the trial would be if the trial ends or I get progression, which is going to be picked up on one of the regular scans and then I am bumped out of the trial and we find out which drug I was actually on. That then decides what is next—whether it is chemo next or whether there is actually another targeted therapy that I can try. Yes, there are very strict guidelines for getting in and there are certainly very strict guidelines—you cannot undertake any other treatments while you are on the trial. But it is better than the alternative.[42]

3.40 In its submission, the NHMRC noted that the criteria for eligibility 'are usually determined by the clinical trial sponsor', such as a pharmaceutical company or a clinical trial network.[43] It was noted that:

> Paradoxically, a sponsor's legitimate aim to reduce confounding factors and thus ensure that a clinical trial produces the highest quality evidence of efficacy, may result in narrow eligibility criteria that significantly lower recruitment.[44]

3.41 Dr Melissa Grady of AstraZeneca explained why inclusion/exclusion criteria are in place:

> It is not an exclusion by want of exclusion. It is simply that, if you follow the science and you want to make sure you have answered that scientific question of that drug or innovative therapy, you must be quite rigorous around the protocol that you design. By virtue of that, it means you have a

40 Mrs Tracy Taylor, *Committee Hansard*, 6 June 2017, p. 9.

41 Ms Simone Leyden, Chief Executive Officer and Co-founder, Unicorn Foundation, *Committee Hansard*, 7 June 2017, p. 15.

42 Ms Nelson, *Committee Hansard*, 6 June 2017, p. 7.

43 NHMRC, *Submission 87*, p. 6.

44 NHMRC, *Submission 87*, p. 6.

> certain population to study and study very well so that you get the right answer at the right time and that you are not wasting time as well.[45]

3.42 On the other hand, the eligibility criterion used by Professor David Thomas of the Garvan Institute for his work in advanced genomics and personalised therapy for people living with incurable rare cancer, is that his patients are unable to access other trials:

> …we have actually designed our modules with exclusion criteria that say: 'The diseases where there are existing trials which people can get access to are excluded from this because there are other trials available. It is the people who do not have the trials available that we are selectively screening.' And we have 170 of those within nine months; we have 600 by the end of this year. There is a huge population that just cannot get access to trials.[46]

Australians in regional and remote areas

3.43 People with cancer in rural and regional Australia also face additional barriers in accessing clinical trials. This was illustrated in the submission received from Mr Denis Strangman AM, whose wife passed away 11 months after her diagnosis with a glioblastoma multiforme grade iv brain tumour:

> …as a general rule, patients from regional centres do miss out, unless they can travel to a major centre. From my knowledge the Canberra Cancer Centre, as an example of a regional centre, has not so far participated in an adult brain tumour clinical trial locally, although some of its patients have – by travelling interstate.[47]

3.44 Ovarian Cancer Australia observed that:

> Patients from rural and regional areas opt out of trials because of the long distances travelled, the cost of travel and finding accommodation and the rigours of travelling while feeling unwell from their illness or the treatment they are undergoing.[48]

3.45 The committee also heard from numerous witnesses about the variations in survival for people living with cancer in regional and remote areas versus those in metropolitan areas.[49]

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45 Dr Melissa Grady, Regional Director, Site Management and Monitoring, Asia Middle East and Africa Clinical Operations, AstraZeneca, *Committee Hansard*, 8 June 2017, p. 25.

46 Professor David Thomas, Director, Garvan Institute, *Committee Hansard*, 8 June 2017, pp 37–38.

47 Mr Denis Strangman AM, *Submission 98*, p. 14.

48 Ovarian Cancer Australia, *Submission 242*, p. 6.

49 See, for example, Mr Harper, CCV, *Committee Hansard*, 18 May 2017, p. 37; Professor Mark Rosenthal, Chair, Cooperative Trials Group for Neuro-Oncology, NHMRC Clinical Trials Centre, University of Sydney; *Committee Hansard*, 18 May 2017, p. 46; Cancer Council Australia (CCA) and the Clinical Oncology Society of Australia (COSA), *Submission 137*, p. 17.

3.46 For example, CCV noted that '65% of people with cancer living regionally survived five years after diagnosis, compared to 69% of people living in a metropolitan Melbourne region', and provided the following table which illustrates the five year survival rate for metropolitan and regional integrated cancer service regions in Victoria, with the regional integrated cancer services highlighted.

Table 5: Five-year survival rates for Victorian Integrated Cancer Services[50]

Integrated Cancer Service	% 5-year survival
Southern	69
Western Central	66
North Eastern	69
Barwon	64
Grampians	64
Loddon-Mallee	65
Hume	67
Gippsland	63
Victorian average	67

3.47 CCV also provided information about the differences in five-year survival for low survival cancers between metropolitan Melbourne and the rest of Victoria, which illustrates that, in many instances, people with LSR cancers living in regional areas have poorer survival outcomes compared with those in metropolitan areas.[51] Table 6 presents this data over a five-year period.

50 CCV, answers to questions on notice, 18 May 2017, (received 18 October 2017), p. 1.

51 CCV, answers to questions on notice, 18 May 2017, (received 18 October 2017), pp 1–2.

Table 6: Five-year survival for low survival cancers between metropolitan Melbourne and rest of Victoria[52]

	% 5-year survival			
Cancer Type	Victoria (whole State)	Melbourne	Rest of Victoria	p-value
Mesothelioma	6	6	6	0.93
Pancreatic	9	10	8	0.02
Cancer of unknown primary	13	13	12	0.14
Lung	18	19	16	<0.01
Liver	19	20	15	<0.01
Gall bladder	23	25	18	0.03
Oesophageal	23	25	20	≤0.02
Central Nervous System (incl Brain)	26	28	22	<0.01
Acute Myeloid Leukaemia	25	25	25	0.90
Stomach	30	31	29	0.12

Funding for travel

3.48 In discussing the barriers to clinical trial participation, the Victorian Comprehensive Cancer Centre (VCCC) submitted that, although the 'largest regional centres can conduct clinical trials, as they have the economy of scale required':

> …a recurring theme in recruiting for clinical trials is that patients from rural and regional areas opt out of trials because of the long distances travelled, the cost of travel and finding accommodation and the rigours of travelling while feeling unwell from their illness or the treatment they are undergoing.[53]

3.49 In his evidence to the committee, Associate Professor Gavin Wright of the VCCC identified the 'regional-rural problem' as the 'No. 1' struggle in recruiting people for clinical trials:

> The kind of surgeon I am is not a common surgeon, so the practice tends to come to me. I look after people from Launceston, Albury-Wodonga, even Adelaide, Mount Gambier and all of Victoria. If I have a trial on at my institution someone from Mildura or somewhere does not get any reimbursement to turn up for a trial presentation. They can only get what limited funding there is from state governments for assistance for actual clinical presentations only, not for turning up to a trial test.[54]

52 CCV, answers to questions on notice, 18 May 2017, (received 18 October 2017), p. 2.

53 Victorian Comprehensive Cancer Centre (VCCC), *Submission 114*, p. 4.

54 Associate Professor Gavin Wright, Research and Education Lead, Lung Cancer, VCCC, *Committee Hansard*, 7 June 2017, p. 41.

3.50 Indeed, in advocating for centres of excellence for people with neuroendocrine tumours (NET), Ms Leyden observed that:

> The problem, obviously, is that most of those centres are located in metro areas, and we see a huge burden for regional patients. We run a NETs patient support line, which is just our nurse who works very hard three or four days a week on the telephone, and we see that about 40 per cent of those calls come from regional areas. So what we would foresee is, yes, those patients still need to be actually funded or helped to go and be seen at these centres of excellence...[55]

3.51 The ANZCHOG National Patient and Carer Advisory Group similarly observed that '[w]here a trial is only available interstate, participation requires funding for interstate travel and accommodation', which is 'a huge financial burden for interstate patients', as currently, there is no funding available.[56]

3.52 In his evidence to the committee, Mr Dan Kent of the Australasian Gastro-Intestinal Trials Group, stated that in New South Wales, 'we get $60 a night to travel to a treating centre, and that really does not cover too much. It would be nice if those costs could be encompassed within trials to get regional, rural and remote people in'.[57]

3.53 In contrast, patients participating in a pharmaceutical clinical trial will generally be reimbursed for travel and other costs associated with attending appointments, unless these patients are on a cooperative group or investigator-initiated (that is, non-commercial) trial.[58]

3.54 In its submission, Ovarian Cancer Australia recommended 'expanding medical travel and accommodation reimbursement schemes to include registered clinical trial participation' in order to 'overcome the reluctance displayed by some rural and regional patients who would otherwise be ideally suited to participate in clinical trials'.[59]

3.55 The Cancer Council Australia (CCA) and Clinical Oncology Society of Australia (COSA) identified the lack of financial assistance as a barrier to people living with cancer participating in clinical trials and provided the following information about existing subsidy schemes:

> Financial assistance to support travel for specialist medical services that are not available locally are offered by state and territory governments and administered through public hospitals. Currently, patients who choose to participate in a clinical trial do not qualify for these schemes. For the

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55 Ms Leyden, Unicorn Foundation, *Committee Hansard*, 7 June 2017, p. 13.

56 ANZCHOG National Patient and Carer Advisory Group, *Submission 125*, p. 9.

57 Mr Dan Kent, Past Chair, Consumer Advisory Panel, Australasian Gastro-Intestinal Trials Group, *Committee Hansard*, 18 May 2017, p. 53.

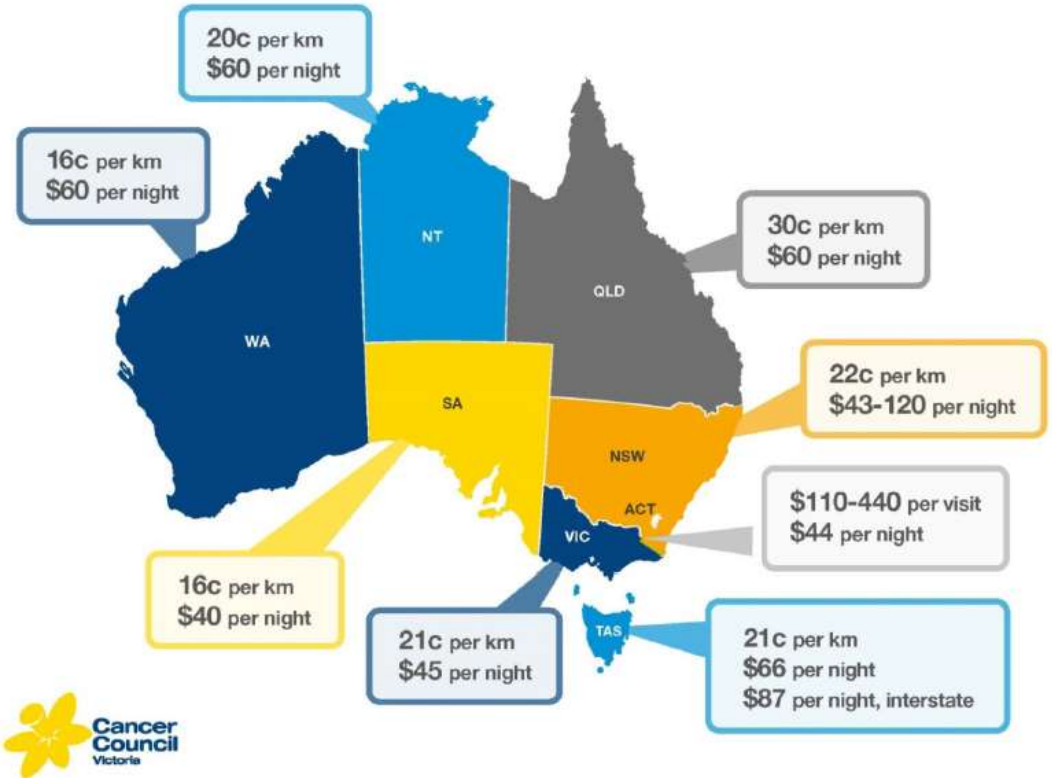
58 CCV, answers to questions on notice, 18 May 2017, (received 18 October 2017), p. 4.

59 Ovarian Cancer Australia, *Submission 242*, p. 6.

> patient, this can reduce their available treatment options and for the researcher, it can limit representation of the rural and remote population in their study.
>
> The various patient travel subsidy schemes lack flexibility to respond to complex circumstances of individual patients, constrain decision making and segregate eligible patients from participating in clinical trials. Additionally, these programs are under-funded and do not meet the real life costs of travel and accommodation. The schemes do not ensure a patient has equitable access to all treatment options regardless of geographic location, and in the interests of the individual and the public, the Government must encourage participation in clinical trials for all cancer patients regardless of geographic location.[60]

3.56 Further, CCV provided the following figure which illustrates the variation in reimbursement for patient transport assistance across Australia.

Figure 8: A comparison of patient travel assistance schemes across Australia[61]



3.57 In order to respond to the barriers experienced by people with cancer in regional Victoria, CCV, together with Cancer Trials Australia and the Victorian Cancer Agency:

> …have funded a three-year project to improve cancer patient access to clinical trials conducted at regional centres. This is one of four projects aiming to implement innovative solutions to increasing patient access to

60 CCA and COSA, *Submission 137*, p. 8 (citations omitted).

61 CCV, answers to questions on notice, 18 May 2017, (received 18 October 2017), p. 3.

> cancer clinical trials. It is intended that the learning from these projects will be applied to improve access to trials at other centres.[62]

The Australian Teletrial Model

3.58 Participation in teletrials is another way in which the barriers facing people living with cancer in regional and remote areas may be ameliorated.

3.59 A 'teletrial' encourages the 'accrual of patients to a suitable clinical trial regardless of geography within a state' by the use of technology to reduce the need for patients to travel to institutions where the trial is taking place.[63] Mr Richard Vines of Rare Cancers Australia opined that '[t]eletrials are the only way that people in the regions…are going to get access to state-of-the-art treatment through clinical trials, if we can somehow build a protocol and manage that remotely'.[64]

3.60 The Australian Teletrial Model, developed by the COSA Regional and Rural Group, and endorsed by the COSA Council:

> …outlines the key considerations for increasing access to clinical trials for people with cancer living in rural and remote communities, and facilitate study activity across rural and remote locations…[and] has the potential to connect research centres, and improve the rate of recruitment to highly specialised clinical trials, including low incidence cancers.[65]

3.61 The Walter and Eliza Hall Institute advocated for the support of the Australasian Teletrial Model, which it submitted would 'encourage accrual of patients to a suitable clinical trial regardless of geography within a state'.[66]

3.62 In their submission, the CCA and COSA provided the following information about the model:

> The model documents a feasible and effective tele-health strategy to increase access to clinical trials closer to home using traditional video-conferencing technology and web based systems. In addition, the model will aid collaboration and networking between centres. This will have a flow on effect for delivering greater engagement in research activity, improving adherence to evidence based practice, improving the rate of recruitment of patients into clinical trials, reducing the disparity in cancer outcomes for geographically dispersed populations, building clinical trial capacity, and providing trial-related training.
>
> Since 2011, utilisation of tele-health in the delivery of services has increased. In the first quarter of the 2011/2012 financial year 1,809 claims

62 CCV, answers to questions on notice, 18 May 2017, (received 18 October 2017), p. 4.

63 Walter and Eliza Hall Institute, *Submission 126*, p. 5.

64 Mr Richard Vines, Chief Executive Officer, Rare Cancers Australia, *Committee Hansard*, 18 May 2017, p. 39.

65 CCA and COSA, *Submission 137*, p. 7 (citations omitted).

66 Walter and Eliza Hall Institute, *Submission 126*, p. 5.

> relating to telehealth services were processed through Medicare compared to 40,570 in the quarter ending 30 June 2016.[67]

3.63 The CCA and COSA suggested the establishment of site specific governance for accredited trial sites in public institutions, to be coordinated at a state and territory or national level, and also supported the Australian Teletrial Model, proposing that:

> …an 'accredited trial site cluster' could be a network of institutions identified as having clinical trials capacity as an established multi-centre collaborative. The level of support provided to the smaller sites would be determined by the complexity of the trial and the clinical capabilities at the site. Increased capacity could be provided from the primary site to potential rural and remote locations through tele-trial models and use of e-technology, such as the Australasian Tele-trial Model.[68]

3.64 To illustrate the way in which the Australian Teletrial Model could operate, the Walter and Eliza Hall Institute, a founding partner of the model, provided the following example:

> …patients in Victoria would have access to a trial open in Victoria at the closest comparable hospital. 'Teleoncology' models of care offer the opportunity for patients living outside major metropolitan centres to access clinical trials closer to home, reducing the need for travel…While the principles of operation for primary and satellite centres are the same, site-specific governance and processes need to be developed for effective implementation.[69]

3.65 Ovarian Cancer Australia also expressed its support for the model.[70]

International trials

3.66 As noted above, the rarity of LSR cancers means that there may not be enough patients in Australia to conduct a stand-alone clinical trial. Indeed, Professor Walker noted that:

> …the barriers to running these trials is actually obtaining numbers for rare cancers, and that is a common thing with all rare cancers. But if you could get all the patients with brain cancer in one centre and available for trials then I think that would accelerate improvements in outcomes. I think that is the difference between here and Europe.[71]

3.67 The effect of the small number of patients with LSR cancers in Australia on the ability to establish clinical trials was also reflected in the evidence of Dr Chris Fraser of the Australian and New Zealand Children's Haematology-Oncology Group (ANZCHOG), who spoke to the importance of

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67 CCA and COSA, *Submission 137*, p. 7 (citations omitted).

68 CCA and COSA, *Submission 137*, pp 6–7 (citations omitted).

69 Walter and Eliza Hall Institute, *Submission 126*, p. 5.

70 Ovarian Cancer Australia, *Submission 242*, p. 5.

71 Professor Walker, *Committee Hansard*, 6 June 2017, p. 49.

international partnerships in continuing to 'provide world's best care for Australian children with cancer':

> That is because our population is very small compared to that of North America or the larger European countries. That means that we really cannot run these clinical trials by ourselves in this patient population; we have to be part of these international collaborative groups. The numbers for each of those trials are becoming smaller and smaller as the subgroups that are eligible for those trials get smaller. For example, for a particularly molecularly targeted drug there is only going to be a small percentage of a certain type of tumour that will be eligible for that trial. So international cooperation and collaboration is increasingly important.[72]

3.68 Dr Fraser noted that he informs his patients about international trials, because '[i]f we do not tell them, the age of the internet is such that they find out about them very quickly':

> It was probably five or six years ago that you could look parents in the eye and say, 'There really is nothing else anywhere in the world other than what we can do here.' That is not the case sitting here today. There are treatments available overseas, some of which have very promising results for very high-risk leukaemias that are proving to be very efficacious.[73]

3.69 However, Dr Fraser also informed the committee about the significant cost of participating in international trials:

> For me to send a patient to North America where they could access one of these trials costs close to $500,000 to $700,000 for them to go and enrol on that trial. That is something that parents now in Australia have the knowledge about and have to deal with. I guess those cells are going to come—they are in clinical trials. We need to position ourselves to be an attractive enough partner that we can participate in those clinical trials, not just in those cellular therapies but other new drugs. It is a rapidly moving field. Our model, which has served us very well, has been to put our hand up to be part of these trials and do it on the cost of the smell of an oily rag. And that just does not work for these new trials. We need to work out a way that we can continue to be attractive partners and continue to have early access in the setting of clinical trials for these new and exciting drugs, so that parents do not have to start looking overseas.[74]

3.70 Indeed, as Professor Terrance Johns identified, access to international trials for Australians was not a regulatory problem, but a funding problem:

> **Prof. Johns:**…Unless the company provides money to specifically do an arm of the trial here or do the trial itself here, they just will not run it. I try to work in that space a bit, but I think we can sell it better. Internationally, I

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72 Dr Chris Fraser, Chair, Australian and New Zealand Children's Haematology-Oncology Group (ANZCHOG), *Committee Hansard*, 7 June 2017, p. 19.

73 Dr Fraser, ANZCHOG, *Committee Hansard*, 7 June 2017, p. 25.

74 Dr Fraser, ANZCHOG, *Committee Hansard*, 7 June 2017, p. 25.

> think we are very competitive, especially with the dollar at 74c. I think it could be very attractive. Americans could do trials here at half the price that they can in the US. I am also on the management committee for COGNO, which is the major body that oversees clinical trials for brain cancer in Australia. We have a very coordinated system across all states in all the major teaching hospitals where we can run these trials; and we do run them, but—
>
> **Senator SMITH:** We could run more.
>
> **Prof. Johns:** we could run more. We certainly have the capacity to run more. It is trying to engage with industry in the US and Europe to come and do some trials here, but we could do more. It is difficult. I applied to do a trial through the new innovation grants, and it got knocked back because they did not see enough value for Australia moving forward. So we are trying to do that.[75]

3.71 The issue of funding was also reflected in Mr Dustin Perry's evidence to the committee:

> There have been times when [the oncologist] has told me that there have been clinical trials running in other countries and they are happy to enrol patients from Australia, but with an international clinical trial, if the principal investigator for that trial is in another country, not in Australia, you are instantly ineligible for government funding. Because a lot of brain cancers, particularly paediatric ones, are so rare, there is not enough of them in Australia to run a meaningful trial at all. The way the funding system is set up literally discriminates against brain cancers and others that are rare.[76]

3.72 Mrs Suzanne Turpie spoke to her frustrations with accessing domestic clinical trials for her son who has brain cancer, when there are trials available overseas:

> We seem to have a standard treatment here depending on the cancer and then an option of a clinical trial; however, if you look overseas, there are options for treatment. Why are those options not available here? Why are those drugs not available here? Why do we have people here in Australia having to crowdfund huge amounts of money—in the hundreds of thousands of dollars—to be able to go overseas to be given the opportunity to fight for their child's survival? They talk to a doctor here and are told: 'There's nothing more that can be done. Go home and wait for your child to die.' This is heart-rending, this is real and this has been said.[77]

3.73 Following the presentation of the above evidence, on 24 August 2017, the Australian government announced that it will co-fund, together with the Robert

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75 Professor Terrance Johns, Director, Brain Cancer Discovery Collaborative, *Committee Hansard*, 18 May 2017, p. 22.

76 Mr Dustin Perry, *Committee Hansard*, 7 June 2017, p. 7.

77 Mrs Turpie, *Committee Hansard*, 18 May 2017, p. 2.

Connor Dawes Foundation, ANZCHOG's AIM BRAIN,[78] 'an international collaborative trial that will enable diagnostic molecular profiling of children with brain cancer'.[79] The duration of the AIM BRAIN is four years, and was accessible from 31 October 2017.[80]

3.74 As discussed in chapter 2, the government also announced on the same date $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'.[81]

3.75 Further, as discussed in chapter 5, on 29 October 2017, the Australian government announced the Australian Brain Cancer Mission, a $100 million fund to defeat brain cancer.[82]

International comparisons

3.76 The following figure illustrates the number of total oncology trials which started between 2007 to 2016, across Australia, China, the US, the United Kingdom (UK), Canada and South Korea.

Figure 9: Phase II/III and III oncology trials, by year of start-up - for China, USA, UK, Canada, South Korea and Australia[83]

3.77 As this figure illustrates, trial activity in China has tripled in less than a decade, and will increase on the basis of the following developments:

78 The Hon. Greg Hunt MP, 'Major new measures to help combat rare cancers', *Media Release*, 24 August 2017, p. 1. This study will be funded through the $79 million of funds available for cancer research, announced in the 2016–2017 Budget.

79 ANZCHOG, *News - Funding for AIM BRAIN announced*, 24 August 2017, http://www.anzchog.org/news/news/2017/08/24/funding-for-aim-brain-announced (accessed 10 October 2017).

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

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

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

83 Medicines Australia, answers to questions on notice, 8 June 2017, (received 20 October 2017), p. 4.

- Firstly, the [Chinese Food and Drug Administration (CFDA)] is actively encouraging the conduct of China clinical studies (including phase I, II and III studies) at the same time as the global clinical trials program; in the past, China studies were inevitably conducted after global programs were largely complete; and
- Secondly, CFDA is actively accelerating the review of Clinical Trial Applications (CTA) and in the last 24 months the number of approvals has increased from 687 (in 2014) to 3666 (in 2016). This is a five-fold increase in just two (2) years across all therapeutic areas; we estimate about half of these approvals are in oncology.[84]

3.78 Medicines Australia informed the committee that '[t]he implication of these developments' is such that:

> …China will start to run more clinical trials as part of global trial programs and that it will recruit quickly. For innovator medicines companies, which must make decisions about where to place trials in the global setting, this means that trials will most likely begin to move from slower and/or more costly markets, to China.[85]

3.79 Ms Elizabeth de Somer of Medicines Australia explained why Australia is no longer as competitive as other countries as a place to run clinical trials:

> …other countries that have entered into the clinical trial competition, such as China, started off at a lower base than Australia and have rapidly met and now exceed Australia's standards. Australian standards have more or less stagnated; we have relied on our quality and we have not improved our costs and time for setting up and initiating clinical trials. These other countries have; they have addressed the issues and then exceeded Australia's benchmark.[86]

3.80 Medicines Australia submitted that the way to overcome these issues would be to establish 'an Australian Office of Clinical Trials to enable a national central point of contact to help drive harmonization and quality standards across the clinical trials sector'.[87]

3.81 Regulatory improvements to clinical trials are discussed later in this chapter.

Committee view

3.82 The committee is concerned by the barriers to accessing clinical trials faced by people with LSR cancers, which appear to be more significant for young people

84 Medicines Australia, answers to questions on notice, 8 June 2017, (received 20 October 2017), p. 3.

85 Medicines Australia, answers to questions on notice, 8 June 2017, (received 20 October 2017), p. 3.

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

87 Medicines Australia, *Submission 141*, p. 3.

and people in regional and remote Australia. The particular challenges for young people with LSR cancers are explored in the following chapter.

3.83 The committee is concerned that there is inconsistency in the availability of trial information for patients through their GPs, and that patients often resort to "Dr Google" to locate information about clinical trials. The committee does not discourage patients from researching possible treatments for their disease, but considers that more could be done to promote the availability of clinical trial information amongst GPs and the public more broadly. The committee notes that, in its evidence to the committee, Cancer Australia conceded that such improvements could be made. Further discussion about how to increase the awareness of GPs and the public of LSR cancers appears in chapter 5.

3.84 The Australian government website, AustralianClinicalTrials.gov.au, has the potential to be a valuable resource to LSR patients and their families. However, the committee has heard that the website is complex and difficult to navigate, requiring those searching to be familiar with precise diagnoses and medical terms. The committee believes that improvements should be made to the Australian clinical trials site so that is a resource and not a further barrier to accessing trials. The CCV's clinical trial website, VCTL, which allows the user to search by cancer type, trial type, phase, molecular target and hospital, and filter results by gender, age, diagnosis, surgical and medical treatment(s) already received, is a much more user-friendly and accessible format. It also provides pop up explanations of medical terms and phrases. In improving the Australian clinical trials website, the Australian government should look to the VCTL as an example.

Recommendation 3

3.85 The committee recommends that the Australian government improves AustralianClinicalTrials.gov.au so it is more accessible and user-friendly.

3.86 The committee appreciates that traditional clinical trial design deliberately excludes certain patients so that results are rigorous and replicable. However, patients with LSR cancers are not your "usual" patients and maintaining the status quo is unacceptable, it is simply hindering progress towards potential treatments and improvements in survival rates. Innovative trial designs must be devised and allowed, with appropriate regulation, to be pursued. The committee welcomes the approach taken by Professor Thomas of the Garvan Institute; the committee encourages more researchers to follow this approach where an exclusion criterion is the availability of other trials.

3.87 While it is not appropriate for the committee or the Australian government to dictate to researchers their scientific methods and protocols, the committee expects that the Australian government will address regulatory barriers which limit the availability of clinical trials for LSR cancer patients. Regulatory barriers are addressed in detail in the following sections of this chapter.

3.88 The committee is also deeply concerned by the difference in access to clinical trials for people with LSR cancer living in regional and remote Australia, in comparison with people living in metropolitan areas. This is particularly egregious

given LSR cancer patients in regional and remote areas suffer worse five year survival rates than their metropolitan counterparts.

3.89 The committee welcomes the Australian Teletrial Model and the national implementation guide issued by COSA.[88] Teletrials will continue to play an important and hopefully greater role in facilitating access to clinical trials by LSR cancer patients in regional and remote areas. However, the committee is of the view that LSR cancer patients in regional and remote Australia must be assisted to participate in person in clinical trials.

3.90 The inability of LSR cancer patients participating in clinical trials to access state and territory patient travel subsidy schemes, and the inconsistency in the subsidies provided, are further barriers to greater participation in clinical trials. The committee urges state and territory governments to consider allowing patients participating in clinical trials to access patient travel subsidy schemes and to agree on consistent subsidy rates based on the distance and method of travel, and the average cost of accommodation in the city in which patients are participating in the trial.

Recommendation 4

3.91 The committee recommends that state and territory governments consider:

- **allowing low survival rate cancer patients participating in clinical trials to access patient travel subsidy schemes; and**
- **agreeing on consistent subsidy rates based on the distance and method of travel, and the average cost of accommodation in the city in which the patient is participating in the trial.**

3.92 Finally, in respect of international trials, the committee welcomes the participation of Australian people with LSR cancers in international clinical trials, and is encouraged by evidence received about the number of participants in such trials. The committee acknowledges that not only does this have a significant impact for the individual involved in the trials, but it may also lead to ground breaking advances for people with LSR cancers. However, participation in international trials often comes at great cost to the patient and the committee considers that more could be done to reduce the financial barriers to accessing international trials for all LSR cancers. The committee would also like to see the inclusion of Australian trial sites in collaborative international trials increase.

Recommendation 5

3.93 The committee recommends that Australian governments improve access to international clinical trials for people with low survival rate cancers, including by:

88 COSA, *Australasian Tele-Trial Model: A National Guide for Implementation*, 19 September 2016, https://www.cosa.org.au/media/332325/cosa-teletrial-model-final-19sep16.pdf (accessed 3 November 2017).

- **exploring ways to reduce the financial barriers to accessing international trials to the extent possible; and**
- **further developing the existing capacity for international collaboration on trials.**

Clinical trials and regulatory issues

3.94 A number of submitters and witnesses raised regulatory issues that impede access to trials for patients with LSR cancers.

3.95 For example, Mr Peter Orchard of CanTeen Australia observed that the research being undertaken by individual states and individual hospitals 'is not always well coordinated and not well shared', and therefore advocated for 'a national direction to be laid out and national strategies to be laid down and have funding attached to them, to try and drive changes in behaviour to a more nationally coordinated approach'.[89]

3.96 The Children's Cancer Research Unit (CCRU) described a clinical trial it undertook that took 12 years to be approved.[90] Professor Jennifer Anne Byrne informed the committee that '[a] lot of the delays were regulatory delays', explaining that:

> We would submit an application. It would go to a body based in Canberra that would consider it. It would take a long time for us to get comments back. We would get those comments. We would need to address them. Then there would be another long period. The regulatory process often involves long periods of waiting, during which time you could work on certain things in the laboratory. You can certainly get things ready but you cannot treat a patient. That is an issue that affects clinical trials but also other kinds of research.[91]

3.97 The following sections examine the most prevalent regulatory issues raised during the course of the inquiry, namely:

- barriers to gaining ethics and governance approval;
- the differences between state and territory jurisdictions;
- the differences between private and public hospitals; and
- issues with respect to insurance.

Ethics and governance approval

3.98 Although it acknowledged that 'some changes have been made to streamline ethics approval processes in Australia' for clinical trial processes, the Children's

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89 Mr Peter Orchard, Chief Executive Officer, CanTeen Australia, *Committee Hansard*, 19 May 2017, p. 7.

90 Children's Cancer Research Unit (CCRU), The Children's Hospital at Westmead, *Submission 88*, p. 3.

91 Professor Jennifer Anne Byrne, Head, CCRU, the Children's Hospital at Westmead, *Committee Hansard*, 19 May 2017, p. 7.

Hospital Foundation and Australian Centre for Health Services Innovation noted that 'governance approval processes remain largely unchanged'.[92]

3.99 The Children's Hospital Foundation and Australian Centre for Health Services Innovation outlined the process for obtaining ethical and governance approval for clinical trial research in Australia:

> Prior to conducting a clinical trial in Australia, it is necessary to obtain approval from a Human Research Ethics Committee (HREC) to ensure that the proposed research will be undertaken in compliance with the National Statement on Ethical Conduct in Human Research (2007). After obtaining HREC approval, it is a requirement in most Australian public hospitals and research institutes to obtain governance approval. Governance approval is based primarily on resourcing, budget, legal, contractual, insurance and indemnity issues, and provides approval to conduct the clinical trial under the auspices of the institution.[93]

3.100 It was further noted that:

> Delays in obtaining governance approval of over a year or more have been reported and primarily result from lack of clarity, consistency and transparency of governance processes. These avoidable delays in ethical and governance approvals are themselves unethical. In addition, most institutions choose to wait until ethics approval is granted before commencing governance review. It is essential that the role of the research governance office in an institution be clearly defined and adequately resourced to ensure that approvals can be issued in a timely manner and patients have access to much needed treatment. Furthermore, it is important that research institutions take responsibility for appropriate training and coordination of ethics and governance submission/re-submission processes including provision of resources that appropriately support the investigators wishing to undertake research.[94]

3.101 CanTeen advocated for faster approval processes for clinical trials in hospitals through the introduction of legislation requiring hospitals to be bound by one ethics process, and changes in the hospital governance process, noting that:

> The fact that you have to go and repeat ethics approvals in multiple settings and get governance approval in multiple settings can really slow down the rollout of a trial, and then, if we are talking about international competitiveness, it does not make us internationally competitive with the other research markets around the world.[95]

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92 Children's Hospital Foundation and Australian Centre for Health Services Innovation, *Submission 280*, p. 2.

93 Children's Hospital Foundation and Australian Centre for Health Services Innovation, *Submission 280*, p. 1.

94 Children's Hospital Foundation and Australian Centre for Health Services Innovation, *Submission 280*, p. 2 (citations omitted).

95 Mr Orchard, CanTeen Australia, *Committee Hansard*, 19 May 2017, p. 7.

3.102 Speaking of her personal experience with the clinical trial process, Mrs Carly Gray, whose young son passed away as a result of a diffuse intrinsic pontine glioma (DIPG), called for a national network of trials across jurisdictions and collaboration between hospitals and research institutions, asserting that '[p]atients cannot afford to wait for trials to begin'.[96]

State and territory jurisdictions

3.103 In respect to ethics approval, the NHMRC observed that:

> The operation of ethics committees and the approval, conduct and monitoring of research are the responsibility of the states and territories that apply both national and state specific guidelines and legislation.[97]

3.104 The NHMRC therefore noted that although it 'is responsible for setting the national standards for human research in Australia', such as the *National Statement on Ethical Conduct in Human Research (2007)*[98] and the *Australian Code for the Responsible Conduct of Research*:[99]

> The authorisation of human research at a particular institution (e.g. hospital or university) and the conduct of that research by a researcher or health practitioner are subject to a variety of national, state and territory laws and policies.[100]

3.105 This variance in laws and policies across jurisdictions was discussed by a variety of submitters and witnesses, who noted a lack of consistency between states and territories with respect to clinical trials.

3.106 In its submission, Medicines Australia recognised that '[t]he systems under which clinical trial sites in Australia are approved differ between states and territory' and the possible difference between sites within states for research governance, is 'an avoidable inefficiency'.[101] It recommended implementing previous recommendations made to the government,[102] as well as:

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96 Mrs Carly Gray, *Submission 116*, p. 3.

97 NHMRC, answers to questions on notice, 29 August 2017, (received 19 September 2017), p. 2.

98 NHMRC, *National Statement on Ethical Conduct in Human Research (2007) - Updated May 2015*, 7 June 2017, https://www.nhmrc.gov.au/guidelines-publications/e72, (accessed 10 October 2017).

99 NHMRC, *Australian Code for the Responsible Conduct of Research*, 8 November 2016, https://www.nhmrc.gov.au/guidelines-publications/r39, (accessed 10 October 2017).

100 NHMRC, answers to questions on notice, 29 August 2017, (received 19 September 2017), p. 2.

101 Medicines Australia, *Submission 141*, p. 6.

102 Namely, implementing 'a combined 30-day best practice benchmark for both ethics and research governance reviews' recommended in 2011 by the Clinical Trials Action Group, and fully implementing the NHMRC 'Good Practice Process' in respect of 'the site assessment and site authorization phases of clinical trials research governance': Medicines Australia, *Submission 141*, p. 6.

> Establishing an Australian Office of Clinical Trials, being a national coordination unit, to enable a national central point of contact to help drive harmonization and quality standards across the clinical trials sector; this would entail working collaboratively with the Commonwealth, States and Territories[103]

3.107 Medicines Australia also outlined the effects of this on GPs and patients:

> Physicians need to have that real-time ability to find out where trials are happening for their patients sitting there right in front of them today. But, because it is fragmented across institutions and jurisdictions, it is very difficult for them to do that, and, because of the way that our primary care and our tertiary care operate, they do not have the time to dedicate to searching for those things.[104]

3.108 The NHMRC outlined the work it has undertaken to streamline clinical trials: between 2013 and June 2017, $6.3 million was provided to the NHMRC under two budget measures, *Expediting Clinical Trial Reform in Australia* and *Simplified and Consistent Health and Medical Research*, 'to develop a nationally consistent approach to clinical trials, improve efficiency and streamline administration and costs with the aim of positioning Australia as a world leader in clinical research'.[105]

3.109 A key outcome resulting from this funding was a National Good Practice Process, piloted at 16 clinical trial sites across all Australian jurisdictions except the Northern Territory, and intended to streamline clinical trial site assessment and authorisation phases.[106]

3.110 As part of its work streamlining clinical trials, the NHMRC also noted that it launched AustralianClinicalTrials.gov.au in 2012, in conjunction with the Department of Innovation, Industry and Science.[107]

3.111 In examining some of these measures, the CCA and COSA commented that '[c]urrent governance and ethics requirements are administratively burdensome and resource intensive, and take considerable time to satisfy'.[108] It was submitted that the structural barriers to conducting clinical trials—which the CCA and COSA consider the 'greatest obstacles to conducting clinical trials in low incidence and low survival cancers', rather than lack of funding—could be overcome by '[i]mplementing systematic changes to improve collaboration will support the sustainability of the cancer research sector and translation of outcomes into practice'.[109]

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103 Medicines Australia, *Submission 141*, p. 5.

104 Ms de Somer, Medicines Australia, *Committee Hansard*, 8 June 2017, p. 23.

105 NHMRC, answers to questions on notice, 29 August 2017, (received 19 September 2017), p. 6.

106 NHMRC, answers to questions on notice, 29 August 2017, (received 19 September 2017), p. 6.

107 NHMRC, answers to questions on notice, 29 August 2017, (received 19 September 2017), p. 6.

108 CCA and COSA, *Submission 137*, p. 6.

109 CCA and COSA, *Submission 137*, p. 5.

3.112 Roche Products Pty Limited (Roche) also identified areas where improvements could be made with respect to streamlining clinical trials. Roche commented that, in Australia, '[m]any approval systems remain inefficient and manual, with wide variation and incompatibility between states and even hospitals within the same state'.[110] Roche continued:

> Governance approval by institutions is often delayed due to inconsistent requirements, based on a poor understanding of essential and non-essential steps. These issues are compounded for rarer cancers where the need to find patients and the lack of treatment centres with expertise may mean ethics and governance delays have a greater impact.
>
> The need for reform has been recognised by many reviews and government committees, including the 2013 McKeon Review of medical research. The Government has committed to addressing competitiveness through an election announcement of $7 million to improve access to clinical trials in Australia and through the [Council of Australian Governments] Health Council. Roche supports urgent action to position Australia as an international research partner of choice.[111]

3.113 Roche therefore recommended that the Australian government '[i]mplement regulatory reforms in partnership with state and territory governments to streamline the clinical trials approval processes'.[112]

3.114 Similarly, the Walter and Eliza Hall Institute discussed the requirement to obtain multiple ethical approvals across states, and made some recommendations for harmonising ethics committees and streamlining governance:

> The time spent obtaining multiple ethical approvals in order to put Australian patients with the same disease on the same trial in different states causes critical delays, with impact on patients' opportunities to receive treatment. Harmonisation of human research ethics committees at a national level should be facilitated. Similarly, governance needs to be streamlined.[113]

3.115 The NHMRC also noted other activities that it has undertaken in order to streamline ethics approval:

- **single ethics review/ 'mutual acceptance'**: the *National Certification Scheme of Institutional Processes related to the Ethical Review of Multi-centre research* commenced in 2010, and NHMRC has certified 44 institutions under this scheme. Additionally, Departments of Health in all states and territories, bar the Northern Territory and Tasmania, are party to an Memorandum of Understanding for the National Mutual Acceptance 'of ethics and scientific review of clinical trials conducted in each of the participating jurisdictions'

110 Roche Products Pty Limited (Roche), *Submission 124*, p. 7.

111 Roche, *Submission 124*, p. 7 (citations omitted).

112 Roche, *Submission 124*, p. 7.

113 Walter and Eliza Hall Institute, *Submission 126*, p. 5.

public health organisations', which is restricted to mutual acceptance between approved state health organisation Human Research Ethics Committees;[114] and

- **the Human Research Ethics Application**: this replaces the National Ethics Application Form (NEAF), and aims 'to facilitate efficient and effective ethics review for research involving humans (i.e. not limited to clinical trials).[115] It was adopted by the IT platform currently used by the health systems in New South Wales, Victoria, South Australia and the Australian Capital Territory 'for the management of ethics review and site approval and authorisation'; however, 'timelines for ethics approval may still vary both within and between the public and private health sectors'.[116]

3.116 Regardless of these changes, the committee heard that in practice, ethics approval is not straightforward. For example, speaking to the time it takes to set up a clinical trial, Mrs Helen Aunedi of Roche informed the committee that 'it comes down to delays in our budget and contractual negotiations', noting that there are '200 accredited ethics committees in Australia'.[117] Mrs Aunedi advocated for 'a centralised committee that can review and approve these clinical studies so we can start quicker', but noted that there is also a delay at the site level, because of the contract, the indemnity and the insurance:

> These are all core templates, so we do not really understand why the institutions are spending so much time negotiating on these issues. But I think, simply, if we could fix that aspect, and then we could perhaps use the national office to promote more of this mutual acceptance. We already have it in place. We just need to have it at the federal level. So it would be great to get support from this inquiry to be able to move that forward.[118]

Public versus private hospitals

3.117 The differences between states and territories in respect of ethics approval and conducting clinical trials also arise in respect of public versus private hospitals.

3.118 For example, Ms Emma Raymond of Wesley Medical Research informed the committee that the process for ethics approval in a private hospital is far simpler than the process in public hospitals:

> In the private sector, you know who your ethics committee is. It is very simple: you know where the forms are, you submit them, and it is done. If they have any questions they will come and ask you. When it goes across to the public system, they have a thing called a NEAF, which is supposed to

114 NHMRC, answers to questions on notice, 29 August 2017, (received 19 September 2017), p. 5.

115 NHMRC, answers to questions on notice, 29 August 2017, (received 19 September 2017), p. 6.

116 NHMRC, answers to questions on notice, 29 August 2017, (received 19 September 2017), p. 6.

117 Mrs Helen Aunedi, Country Head, Country Clinical Operations, Roche, *Committee Hansard*, 8 June 2017, p. 23.

118 Mrs Aunedi, Roche, *Committee Hansard*, 8 June 2017, p. 23

> allow for an easy application—one large application, and then site-specific applications for each hospital. But it does not work that way. I did a NEAF that was approved—one site was approved. I used the same documents for another Queensland Health hospital and we had to rewrite everything …
>
> A NEAF…has about 61 pages where you answer a lot of questions and upload documents about the research, and then, for each hospital site that you want access to, you then have to do another application, which is then looked at by each hospital's ethics committee. Once it is approved there, it then goes to the governance committee. The problem arises if you have not filled something out correctly. At one point I had the wrong number on a page. They do not tell you that; they just put it on hold and then when they finally get back to you have to resubmit it again, but you have missed the next deadline for the ethics committee, so then it gets held over again and then, if it gets to governance, and they do not like the paperwork, it gets held up again. That is before you even start the research.[119]

3.119 Ms Raymond also observed that there are different time pressures on clinicians in public, compared to private, hospitals:

> …in the private sector there is more of a focus on clinician research. In the public sector they are too busy and there are too many people involved from start to finish. Sometimes, the clinician who is doing the care will not even know that they have gone on to have treatment because it is just such a busy, fast-paced scenario.[120]

3.120 Ms Delaine Smith of the ALLG informed the committee that private institutions 'are traditionally not substantial contributors to investigator initiated clinical trials', explaining:

> There is little to almost no incentive for private facilities or clinicians to have their patients participate in clinical trials. This impacts adversely on the rate of patient accrual to clinical trials. The second point is that, additionally, there is no incentive or support from private health insurers to have their patients participate in clinical trial research—it is simply not there. One could argue that it is even a greater priority for the private sector to participate and champion research that inevitably will have the potential to bring about healthcare efficiencies and cost savings.[121]

3.121 Professor Andrew Roberts, also of the ALLG, provided a further explanation:

> It is quite clear that to be involved in a clinical trial requires extra care, extra time, extra resources and therefore extra costs. Clearly that affects issues around reimbursement, whether that is through private or government. Ultimately, to participate in clinical research, the patient, the doctor, the sponsor of the trial and our health system are invested, and it is a

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119 Ms Emma Raymond, Theme Leader, Cancer, Wesley Medical Research, *Committee Hansard*, 6 June 2017, pp 29–30.

120 Ms Raymond, Wesley Medical Research, *Committee Hansard*, 6 June 2017, p. 33.

121 Ms Delaine Smith, Chief Executive Officer, ALLG, *Committee Hansard*, 7 June 2017, p. 30.

> question of whether they are clear about that and whether there is an alignment of purpose.[122]

3.122 The ALLG suggested that the way in which to overcome the obstacle that clinicians are time poor, which can impact matters such as timely access to information about clinical trials, could be to encourage models that encourage public/ private partnerships.[123] The ALLG also recommended enabling collaboration between public and private institutions by engaging with insurance companies and the private health care sector, and implementing 'national clinical trial uptake across public and private hospitals' as ways to improve survival rates by establishing Key Performance Indicators (KPIs) for hospitals regarding clinical trial participation, their uptake of patients to clinical trials, and creating 'a culture of positive benefit'.[124]

3.123 CanTeen Australia also proposed collaboration across institutions, recommending the establishment and operation of national low survival cancer trial networks which would:

> …operate across multiple hospital boundaries (including across local health districts, public and private hospitals and adult and paediatric settings), assure rapid trial initiation, consistent, cost effective and timely ethics, governance and other relevant approvals, rapid and targeted access to patients and consistent monitoring processes and standards.[125]

Insurance

3.124 In evidence to the committee, CanTeen highlighted the importance and benefits of exploring options around a national insurance scheme covering clinical trials which would alleviate the burden that individual hospitals currently face by having to seek coverage for a given trial:

> … in terms of insurance: again, could there be a national insurance scheme that covers trials so that we do not have this business of every hospital having to go to see whether their particular insurer will cover them for this trial?
>
> Just in terms of that insurance process alone: that gets replicated in every hospital, let alone them needing to ask about the impacts on their staffing or their budget. It is an understandable process that they have to do, but to take four or five months for it is the part that does not seem to be valid, really. If we are really keen about getting patients into trials quickly and getting good research happening, we need to make those times shorter.[126]

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122 Professor Andrew Roberts, Member and Director, ALLG, *Committee Hansard*, 7 June 2017, pp 30–31.

123 ALLG, *Submission 121*, p. 2.

124 ALLG, *Submission 121*, p. 5.

125 CanTeen Australia, *Submission 128*, pp 4–5.

126 Mr Orchard, CanTeen Australia, *Committee Hansard*, 19 May 2017, p. 7.

3.125 In response to this suggestion, Professor Anne Kelso of the NHMRC stated that it was outside of the NHMRC's remit to do such work, and that 'unless we were tasked and funded to do a particular project; it's otherwise not within the remit of NHMRC's activities'.[127]

Committee view

3.126 While there have been recent changes to improve streamlining of clinical trial ethics approval, the evidence presented to the committee indicates that differences in ethics and governance approval processes between states and territories, and private and public hospitals continue to delay and in some instances discourage trials or trials across multiple sites.

3.127 The committee welcomes suggestions from various submitters and witnesses, such as removing the requirement to obtain ethics and governance approval for each individual trial site; the establishment of an Australian Office of Clinical Trials to be a national coordination unit and national central point of contact to help drive harmonization and quality standards; further regulatory reforms to streamline approvals processes; and facilitating better collaboration between private and public institutions.

3.128 The committee recommends that Australian governments address the remaining barriers arising from differences in ethics and governance approval processes as a matter of priority, and in doing so give serious consideration to the proposals recommended to this inquiry.

Recommendation 6

3.129 The committee recommends that Australian governments, as a priority, further streamline ethics and governance approval processes for clinical trials, particularly where those processes differ between states and territories, and public and private research institutions.

3.130 Further, the committee acknowledges the work that the NHMRC has done to reduce unnecessary regulatory barriers with respect to ethics processes, and while it recognises that some processes are beyond the scope of the NHMRC, the committee considers that the NHMRC could make further changes in order to eliminate those existing, significant regulatory delays.

3.131 Specifically, the committee considers that the NHMRC could develop a standard template and associated guidelines, including timeframes, for ethics and other governance approvals that could be adopted by every state and territory. This in turn could allow for the approval from one institution to lead to automatic approval at any other institution.

127 Professor Anne Kelso, Chief Executive Officer, National Health and Medical Research Council, *Committee Hansard*, 29 August 2017, p. 30.

Recommendation 7

3.132 The committee recommends that the National Health and Medical Research Council develops a standard template and associated guidelines, including timeframes, for ethics and other governance approvals for consideration and possible adoption by each state and territory.

Chapter 4

Paediatric and youth cancers

4.1 This chapter examines low survival rate (LSR) cancers that affect children and young people.

4.2 Cancer Australia defines a child 'as a person aged less than 15 years', and provides the following information about cancers in this age group:

> The types of cancers that occur in children, and the way they respond to treatment, can be different from cancers that occur in adults. They can also be different from the types of cancers that occur in adolescents and young adults (aged 15–29 years) – there are often specific protocols and guidelines for the management of adolescents and young adults with cancer, which bridge the gap between children's cancers and adult cancers.[1]

4.3 However, Mr Peter Orchard of CanTeen Australia explained that the definition of a child varies across jurisdictions:

> In [Western Australia] there is a hard line drawn that will come into play in the next few months—when a young person turns 16, they are then directed to the adult setting even if they have been treated in the paediatric setting. In Victoria, with the Royal Children's Hospital, there is more flexibility; they will go up to 18. So there are just two examples of the extremes.[2]

4.4 Cancer Australia also provided an explanation of why cancer occurs in children:

> In most cases, we don't know why children get cancer. Children are too young to have the same risk factors that affect adults (e.g. environmental exposures, lifestyle, infections). Tumours occasionally develop as a result of a genetic error made in children's growing bodies.
>
> …
>
> In children, age is not a risk factor for cancer, but the incidence of some cancers varies with age. Some childhood cancers tend to appear in very young children and others in older children. Family history is also important because a few childhood cancers run in families.[3]

4.5 The following sections examine the most common LSR cancers in this group, the unique issues and challenges faced by this group of people with LSR cancer and the difficulties with transitioning from paediatric to adult treatment and care. Prior to

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1 Cancer Australia, *About children's cancer*, https://childrenscancer.canceraustralia.gov.au/about-childrens-cancer/what-childrens-cancer (accessed 4 October 2017).

2 Mr Peter Orchard, Chief Executive Officer, CanTeen Australia, *Committee Hansard*, 19 May 2017, p. 3.

3 Cancer Australia, *About children's cancer*, https://childrenscancer.canceraustralia.gov.au/about-childrens-cancer/what-childrens-cancer (accessed 4 October 2017).

a discussion of these issues, the section below considers the personal impact of childhood and youth cancers.

The personal impact of childhood and youth cancers

4.6 Many parents, relatives and friends of children and young people who have suffered from or are currently diagnosed with cancer shared their experiences with the committee. Childhood and youth cancer have a devastating effect on the child or young person with cancer, their family and their community:

> The impact of a child dying is pervasive. It is not just the adults who cannot rationalise the injustice of it; it is also the children—siblings, cousins, and friends. They are all suddenly faced with their own mortality because something they rationalise as being for the elderly has happened to one of their peers. While we as adults continue to grieve, so do the children—nightmares, bedwetting, anxiety, and withdrawal. It goes against nature. Parents are not supposed to outlive their children. Children are not supposed to be diagnosed with diseases devoid of survival rates. We should be able to reassure children that doctors can help them, not have them living in fear that if they were to get brain cancer they would end up like Tom and the 34 other Australian children who die from it each year.[4]

> As a parent of a child who has been diagnosed with brain cancer – words can be hard to muster to describe how this has impacted our family. It is devastating. It is all consuming. It is heartbreaking.
>
> …
>
> Brain cancer seems to offer one blow after another. We don't make plans. The plans we do make we often cancel. Life becomes a circle around appointment times and there is not much left in the way of finances or energy for normal social life.[5]

> We reside in country Victoria and, while I have spent time with Chloe while she has been in hospital in Melbourne, I have been on constant call to care for her brother and sister often without notice. I have had to try and find a way to calm their fears when their sister is so ill and they desperately want her and their mum and dad to come home. Not only have I had to watch my precious granddaughter in such pain and going through horrendous treatments as well as seeing the hurt and worry of her mother and siblings, I have had to watch helplessly as my younger son struggles through his emotional pain knowing there is nothing he can do to make his little girl better. This is heartbreaking for me. A parent is supposed to be able to protect their children from pain and hurt.[6]

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4 Mr Simon Gray, *Committee Hansard*, 7 June 2017, p. 4.

5 Mrs Tracy Taylor, *Submission 52*, p. 1.

6 Ms Elizabeth Perry, *Submission 78*, p. 1.

> I cannot put into words the suffering our precious daughter Brooke endured, and now for my wife Olivia and I continue that suffering every second of every day. We celebrated Brooke being one of the lucky 1 in 5 survivors of Brain Cancer only to have her taken from us by this hideous disease 10 years later.[7]

> When I was 18, I was diagnosed with Gastro Intestinal Stromal Tumor [sic] (GIST) and was told by my disease had no cure and I was likely to have about one year left to live. There was no cure in 1996. There is still no known cure 21 years later. GIST is a rare cancer with low survival rates.[8]

> In September 2016 our 13 month old daughter, Isabella, was diagnosed with brain cancer. She has a grade 3 anaplastic ependymoma. It is an aggressive cancer; the most aggressive form of ependymoma. This insidious disease took over ¼ of our daughter's brain before she was diagnosed. Instead of our family watching our little girl transition from a baby to a toddler, witness her first wobbly steps, hear her learning to talk, we watched her literally fight for her life. Over the course of a week, the longest and most awful week of our lives, we stood by while Isabella endured 4 brain surgeries. We watched her suffer countless seizures, the last one requiring a MET call with staff from the ward, PICU and Emergency attending to assist to try to stabilise her. We watched as infection racked her body forcing her temperature up to 40 degrees. We watched as a ventilator breathed for her. We waited helplessly every time she was taken away to the operating theatre, not knowing if she would return to us. We listened to the neurosurgeon tell us that he had to abandon the surgery to debulk her tumour because of massive blood loss. We listened as he told us that they transfused the entire volume of blood in her body 3 times over before she was able to be stabilised. We cried when she finally woke up and said "mummy", "daddy" and "happy" (her 3 favourite words). We cried when we realised she was paralysed down her right side. We cried when we realised she could not swallow, could not eat, could not drink and could not sit up. We cried when she went mute several days after her fourth surgery. We cried a lot that week. We still cry a lot now.[9]

4.7 In addition to the emotional toll of these cancers, there are broader implications. For example, in respect of brain cancer, Love for Lachie submitted that:

> Most parents will be unable to work when their child is diagnosed with brain cancer as they need to care for their child fulltime throughout surgeries, radiation, chemotherapy and other treatments. Brain cancer is the undisputed most financially costly cancer. Parents can not work if they have

7 Mr Jonathan Karl Fretwell, *Submission 99*, p. 3.

8 Mrs Sarah McGoram, *Submission 159*, p. 1.

9 Ms Robin Berthelsen, *Submission 170*, p. 1.

> a child diagnosed; adults who are diagnosed can no longer work; treatment options that are not part of the gold standard treatment plan are incredibly expensive and for many people become completely financially prohibitive leaving them to accept their fate with standard ineffective treatment.[10]

4.8 Some of these broader effects of LSR cancers, such the loss of income, are discussed in chapter 5.

LSR cancers most commonly affecting children and young people

4.9 There are a range of LSR cancers that commonly affect children, for example, Cancer Australia identified the following cancers: leukaemia, brain and other central nervous system tumours, Hodgkin disease (Hodgkin lymphoma), non-Hodgkin lymphoma, neuroblastoma, soft tissue sarcoma, kidney tumours, melanoma, bone tumours, germ cell tumours, retinoblastoma and liver tumours.[11]

4.10 The committee heard from various submitters and witnesses that brain cancer kills more Australian children than any other disease,[12] and while 'the overall survival of some children with brain tumours has improved' in the paediatric setting, 'the groups of children with poor outcomes are becoming smaller, and therefore increasingly challenging to study'.[13]

4.11 The Australian and New Zealand Children's Haematology-Oncology Group (ANZCHOG) made a similar observation:

> Childhood cancer comprises less than 1% of the total number of new cancer diagnoses in Australia each year. This equates to more than 600 children diagnosed with cancer each year. The treatment of childhood cancer is one of the great success stories of modern medicine. Survival rates have increased from less than 30% in the 1960s to 80% in the 2000s for all childhood cancers combined. For Acute Lymphoblastic Leukaemia (ALL), the most common form of childhood cancer, the cure rate now approaches 90%. Despite these outstanding successes childhood cancer remains the leading cause of non-accidental death in children in Australia and many subtypes of childhood cancer continue to have a very poor prognosis. Unfortunately, the rate of improvement in survival for children with cancer has plateaued over the past decade.[14]

4.12 CanTeen Australia identified that cancer in adolescents and young adults (AYAs) 'has a distinct biology and responds differently to treatments that are

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10 Love for Lachie, *Submission 120*, p. 7.

11 Cancer Australia, *Types of children's cancers*, 23 August 2015, https://childrenscancer.canceraustralia.gov.au/types-childrens-cancers (accessed 4 October 2017).

12 See for example, Brain Cancer Discovery Collaborative, *Submission 60*, p. 1; Love for Lachie, *Submission 120*, p. 1; Children's Hospital Foundation, *Submission 274*, p. 2.

13 Children's Cancer Research Unit (CCRU), *Submission 88*, p. 4.

14 Australian and New Zealand Children's Haematology-Oncology Group (ANZCHOG), *Submission 237*, p. 2.

otherwise successful in paediatric or older adult populations'.[15] In respect of survival rates for AYAs, CanTeen Australia stated that:

> Although overall survival rates are good…at approximately 88%1, this masks poorer outcomes seen in several high lethality cancers for this age group. Five-year survival for cancers such as Acute Myeloid and Acute Lymphoblastic Leukaemias and Brain and Bone cancers are still exceptionally low at between 61.3% and 65.6% with Sarcoma only slightly higher at 76.7%, with others such as Rhabdomyosarcoma and Lung and Adrenocortical Carcinomas having 5 Year survival rates well below 40%, and Hepatic Carcinoma only 20.6%.[16]

Unique challenges and issues

4.13 The committee heard from a number of parents and professional organisations about the particular challenges and issues faced by children and young people with cancer.

4.14 For example, The Kids' Cancer Project stated that '[t]he challenges of new anti-cancer drug development for childhood cancers that are faced globally are exacerbated in Australia because of our relatively small population'.[17] These challenges generally arise because of 'the rare nature, smaller population, limited access to tumour samples, more limited bodies of research knowledge and therefore reduced funding opportunities'.[18]

4.15 The Kids' Cancer Project also noted that '[w]e have seen the improvement in prognosis of several [childhood] cancers that have had dedicated, focussed funding from the Federal government', but:[19]

> The rarity of several childhood cancers means that they are not covered by the burden of the population which the current National Health and Medical Research Council [(NHMRC)] funding model is based on.[20]

4.16 The Children's Cancer Research Unit also discussed challenges arising from the NHMRC funding model, asserting 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

15 CanTeen Australia, *Submission 128*, p. 3 (citations omitted).

16 CanTeen Australia, *Submission 128*, p. 2 (citations omitted).

17 The Kids' Cancer Project, *Submission 136*, p. 3.

18 The Kids' Cancer Project, *Submission 136*, p. 5.

19 The Kids' Cancer Project, *Submission 136*, p. 2.

20 The Kids' Cancer Project, *Submission 136*, p. 3.

> 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".[21]

4.17 Indeed, clinical trials were identified by The Kids' Cancer Project as 'the single most important factor contributing to the dramatic improvements in survival rates for children with cancer over the past forty years'.[22]

4.18 In speaking of access to clinical trials for children, Dr Chris Fraser of ANZCHOG noted that:

> The fact that childhood cancer is relatively rare in one way assists our ability to conduct clinical trials because the care is very centralised. Essentially, all of these children are cared for in one of eight children's cancer centres around the country.[23]

4.19 However, ANZCHOG raised a number of obstacles to running clinical trials, including the expense of clinical trials, reluctance by pharmaceutical companies to run trials in Australia due to the small population size, and accessing targeted drugs.[24]

4.20 The importance of clinical trials focussed on children and young people was similarly emphasised by CanTeen Australia, which noted that AYAs face particular challenges:

> Compared to paediatric and older adult populations, AYAs have experienced relatively poorer survival gains and reductions in mortality, in part driven by poorer access to clinical trials. Embedding clinical research within standard paediatric care has been the single most important driver of the dramatic improvements in childhood cancer survival rates seen over the past 40 years. Compared with the approximately 45% of younger children with cancer in Australia who currently participate in potentially lifesaving clinical trials, AYA participation rates remain low at approximately 10%.
>
> The rarity of some cancers which disproportionately impact this age group is another reason for the poorer improvements in length of survival and mortality. Despite improvements in the diagnosis and treatment of common cancers that have resulted in dramatic reductions in mortality, early

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21 CCRU, *Submission 88*, p. 2.

22 The Kids' Cancer Project, *Submission 136*, p 3. See also ANZCHOG National Patient and Carer Advisory Group, *Submission 125*, p. 6.

23 Dr Chris Fraser, Chair, ANZCHOG, *Committee Hansard*, 7 June 2017, p. 19.

24 ANZCHOG, *Submission 237*, pp 4–5.

> diagnosis programs for rare cancers have not improved over the last 20 years and diagnosis often remains slow, resulting in the cancer being diagnosed at a more advanced stage.
>
> In addition, rare cancer treatments have not advanced at the same pace as those for common cancers and it is likely that many patients with rare cancers are receiving suboptimal care; hence a rare cancer diagnosis is often accompanied by a very poor prognosis. AYAs diagnosed with a rare cancer are significantly more likely to die from their disease, with these cancers being responsible for the majority of cancer-related deaths in this age group.[25]

4.21 Further, CanTeen Australia submitted that, in circumstances where people experience paediatric cancers in their 20s:

> …ideally they should be able to be part of a paediatric trial. We forget the fact that it is a paediatric trial; what we do remember is that it is a trial in this particular topic cancer. If they have got that type of cancer, they should be able to be part of it.[26]

4.22 The difficulty faced by young adults was also noted by ANZCHOG, which stated that the issue of eligibility for clinical trials for young people between the ages of 14 and 18 'is a bit of a grey area'.[27] Dr Fraser elaborated:

> Adolescents and young adults have some poorer outcomes in some types of cancers, and they are not enrolled as frequently on clinical trials. There is also a discrepancy sometimes between the treatment the same patient with the same sort of cancer might receive in a paediatric institution compared to in an adult institution. And there might be discrepancies between the treatment they might receive in a private adult institutions and a public institutions, for example.[28]

Transitioning to adult treatment

4.23 The committee heard that there are particular challenges faced by cancer patients who transition from paediatric to adult treatment and care. For example, CanTeen Australia informed the committee about the 'disruption to treatment' experienced by these patients:

> If they are having treatment and then at 16 they have to be bumped across to a new institution, a whole new team needs to pick them up at that point. In terms of research, it is that, by definition, they are still a child but they are not able to be part of a paediatric trial because they are considered to be too old for a paediatric setting. And the hard rule around paediatric trials is that they have to happen in a children's hospital that has been approved by [the Children's Oncology Group (COG)]. They have teams that go around

25 CanTeen Australia, *Submission 128*, pp 2–3 (citations omitted).

26 Mr Orchard, CanTeen Australia, *Committee Hansard*, 19 May 2017, p. 3.

27 Dr Fraser, ANZCHOG, *Committee Hansard*, 7 June 2017, p. 22.

28 Dr Fraser, ANZCHOG, *Committee Hansard*, 7 June 2017, p. 23.

> the world accrediting hospitals for COG trials, but they will not look at any hospital other than a paediatric hospital. So a 16- or 17-year-old will not be able to participate in the trial because they cannot attend a setting.[29]

4.24 This was also discussed by Professor David Walker:

> **CHAIR:** I understand there is a huge difference, if I can put it that way, in regard to the way children compared with adults get treated for exactly the same disease. So if you are moving from the paediatric area to the adult area it is quite often a bit of a shock. Do you find that?
>
> **Prof. Walker:** There is no doubt about that. In fact, I think that is one of the reasons why the outcomes for children's cancers—for some cancers—have improved to some extent over the years. They get better coordinated care. Their care is centralised, by the way, so therefore a lot of the patients are either available for, or have access to, the latest trials. There is no doubt that there is a greater appetite for coordination of care and longitudinal care in the paediatric medical community compared to adults.
>
> …
>
> **Prof. Walker:** …even young adults, particularly those ones transitioning through: they find they are in between and they do not get either. They do not get the benefit of either.
>
> **CHAIR:** I understand that when you move from being a paediatric patient to AYA you do not have the same team. Is that correct?
>
> **Prof. Walker:** That is true for a lot of things. Kids who have long-term problems lose contact with the team that has been looking after them. Team care is far less applied in adult medicine compared with children's medicine, in a variety of fields. So, yes, it is really quite difficult when kids get older, whether it be brain cancer or other neurological problems like spina bifida and things like that—but we are getting off topic. But that is absolutely true. Absolutely true.[30]

4.25 Clinical Associate Professor Nicholas Gottardo of ANZCHOG also informed the committee that transitioning to adult treatment 'is a bit of an issue', which varies across states, but that:

> …in general, we would not be transitioning a patient during treatment. If we have taken a patient who is 16 or 17 under our care, we will complete the therapy that is prescribed for that particular patient. Then a transition model would be developed with a particular clinician or hospital, depending where that care was best served. So, generally, we would not be transitioning a patient [mid-treatment]. That may occasionally happen as a patient gets well beyond 18 years of age and potentially has a resistant

29 Mr Orchard, CanTeen Australia, *Committee Hansard*, 19 May 2017, p. 3.

30 Professor David Walker, *Committee Hansard*, 6 June 2107, p. 50.

> tumour that is not responding to the treatment that we have delivered up front.[31]

4.26 Clinical Associate Professor Gottardo identified the 'wider issue' for transitioning patients as:

> …having a pathway of coordinated care for a child or an adolescent—or even a child survivor of cancer—into the adult environment, where they are much more left to their own devices, as opposed to the more paternalistic paediatric model where we kind of take care of everything. That type of care can certainly be disjointed. We are now much more aware of this issue and we are setting up transition clinics et cetera to try and have a smoother transition between our service and the adult service.[32]

4.27 Clinical Associate Professor Gottardo acknowledged the evidence received by the committee that some children and young adults 'fall between the gaps', and although it is not a 'major problem' for children up to 16:

> …I think the 16- to 18-year-olds fall between the gaps. Often children's hospitals' business model is younger children, so there are often restrictions on being able to accept children between 16 and 18. Different states have different rules on it. It can also depend on whether the child, or the young adult, ever gets referred to a paediatric centre. Sometimes we just never find out about them, and we may have a clinical trial available.
>
> Many of our clinical trials with the children's oncology group go into their early 20s—some of the sarcoma trials go into their 30s—and we would be able to enrol such patients in a trial. But the adult sector are not part of those oncology groups and therefore would not be able to and may or may not have access to trials. But the data certainly suggests that that is the group that falls between the gaps for enrolling in clinical trials. If they are admitted to a paediatric centre then there is no difference, but if they are admitted to an adult centre then they seem to have very low enrolment in an up-front clinical trial.[33]

4.28 Indeed, Mr Robert Perkins—whose son was 17 at the time he was diagnosed with a GBM malignant tumour and passed away at the age of 21—shared his experience that his son was too old for a children's hospital, and that '[t]here was little or no support for adolescents who are dealing with their own mortality in a hospital system that is mostly dealing with mature adults'.[34]

Committee view

4.29 The committee cannot adequately express its thanks to the individuals who shared their personal experiences of paediatric and youth cancer. The devastation of

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31 Clinical Associate Professor Nicholas Gottardo, Deputy Chair, ANZCHOG; and Chair, Central Nervous System Tumour Subcommittee, ANZCHOG, *Committee Hansard*, 7 June 2017, p. 23.

32 Clinical Associate Professor Gottardo, ANZCHOG, *Committee Hansard*, 7 June 2017, p. 23.

33 Clinical Associate Professor Gottardo, ANZCHOG, *Committee Hansard*, 7 June 2017, p. 23.

34 Mr Robert Perkins, *Submission 184*, p. 1.

cancer is often compounded when a child or young person—who has barely commenced their life—is diagnosed. The committee wants to acknowledge the bravery and resilience of these children and young people, and their families, who in the face of great personal tragedy strive for knowledge and solutions not only for their own benefit but also in a quest to spare other families the same trauma.

4.30 Recommendations elsewhere in this report are applicable to the challenges facing children and young people with cancer; the committee hopes that action is taken so that all people with LSR cancers face improved prognoses in the future and that significant in-roads are made to improve the diagnosis and treatment of all LSR cancers. In particular, the committee hopes that greater financial support for innovative clinical trials, increased flexibility in clinical trial design and access, and improved ethical and governance approvals will see more research into LSR cancers affecting children and young people.

4.31 The committee is concerned about the transition from paediatric to adult oncology care where it appears, at least in some settings, that children are abruptly removed from paediatric oncology services and moved to adult oncology services.

4.32 The committee notes that this change from paediatric to adult oncology services is the responsibility of the state and territory health systems. The committee encourages the states and territories to consider their current arrangements for transitioning children and young people from paediatric to adult oncology services, and ensure that this occurs in a consistent and co-ordinated way that ensures continuity and quality of care in the best interests of each individual patient.

Recommendation 8

4.33 The committee recommends that, through the Council of Australian Governments Health Council, the Australian government leads a process to ensure that arrangements for transitioning children and young people from paediatric to adult oncology services occurs in a consistent and co-ordinated way that preserves continuity and quality of care in the best interests of each individual patient.

Chapter 5

Increasing survival rates for people with low survival rate cancers

5.1 This chapter discusses suggestions put to the committee intended to increase survival rates for people with low survival rate (LSR) cancers. In particular, this chapter considers:

- the importance of early detection and diagnosis;
- data and biobanking;
- genomic medicine and biomarkers;
- access to medicines;
- care and support services for patients and their families; and
- a national strategy on LSR cancers.

Early detection and diagnosis

5.2 The committee heard from a number of submitters and witnesses about the correlation between early detection, screening and diagnosis, and increased rates of survival for people with cancer.[1] The committee also heard from people with LSR cancers, and their relatives, about their desire for early detection of these cancers.[2]

5.3 Professor Guy Eslick spoke about positive developments for the majority of cancers over the last 70 years due to early detection:

> In the 1950s, the majority of cancers—that is about 75 per cent of all cancers—had a five-year survival of about 50 per cent. Only half of them were likely to live five years. Today most of these cancers have had substantial improvements in their five-year survival. There are a number of reasons for this improved survival, including increased research funding, dedicated researchers, early detection and screening programs, education of the public regarding risk factors that can be modified to reduce the risk and, of course, newly developed treatments. However, there are a group of cancers where the survival rates have not changed much at all in the last 70 years, and this is unacceptable.[3]

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1 See, for example, Ms Simone Leyden, Chief Executive Officer and Co-founder, Unicorn Foundation, *Committee Hansard*, 7 June 2017, p. 12; Mr Daniel Goulburn, Member, Pancreatic Cancer Alliance (PCA), *Committee Hansard*, 7 June 2017, p. 49.

2 See, for example, Ms Belinda Peden, *Submission 143*, p. 5; Mrs Lyndall Bates, *Submission 180*; Ms Frances Burrows, *Submission 265*, p. 2.

3 Professor Guy Eslick, Professor of Cancer Epidemiology and Medical Statistics, University of Sydney, *Committee Hansard*, 18 May 2017, p. 56.

5.4 Professor Eslick therefore advocated that the Australian government should focus 'on identifying risk factors, prevention and screening programs for low-survival cancers'.[4]

5.5 The positive effect of early detection was also discussed by Dr Nicola Waddell, who informed the committee that early detection of pancreatic cancer—a LSR cancer that is 'increasing among young females'[5]—would 'mean a larger proportion of patients can undergo surgery',[6] which could lead to an increased chance of survival for these patients.

5.6 In relation to the improvements in breast cancer survival rates, Mr Richard Vines of Rare Cancers Australia (RCA) observed that since 1990, the survival rate has increased from 60 per cent to 90 per cent due to screening, and commented that '[e]arly diagnosis is everything'.[7] Mr Vines also spoke about the importance of public awareness and its role in early detection:

> …how do you tell the public that if they have a pain that does not go away that they should not just take two aspirin in perpetuity but that they should do something about it? Virtually every patient who comes to us has been three or four months in the diagnosis. That is critical because that is the time when the cancer is likely to metastasise. For example, breast cancer patients with metastatic cancers do not do well; you want to understand it early.[8]

5.7 Mr Daniel Goulburn of the Pancreatic Cancer Alliance similarly spoke to the high rates of survival for breast cancer, as well as prostate and colon cancer, and how this correlates with early detection as well as public awareness programs.[9] Mr Goulburn noted that 'there is a general awareness amongst the general public and good education of frontline medical practitioners' of such cancers when compared with pancreatic cancer, which currently has a survival rate of 7.7 per cent: a marginal improvement over the last 30 years.[10]

5.8 Other witnesses also raised the lack of awareness of LSR cancers amongst GPs, which hinders early detection and diagnosis. For example:

> **CHAIR:** …I want to ask you how you feel awareness is amongst GPs and other medical practitioners. Do you feel there should be an awareness campaign for them?

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4 Professor Eslick, University of Sydney, *Committee Hansard*, 18 May 2017, p. 56.

5 Professor Eslick, University of Sydney, *Committee Hansard*, 18 May 2017, p. 56.

6 Dr Nicola Waddell, Group Leader, Medical Genomics Group, QIMR Berghofer Medical Research Institute (QIMR Berghofer), *Committee Hansard*, 6 June 2017, p. 38.

7 Mr Richard Vines, Chief Executive Officer, Rare Cancers Australia (RCA), *Committee Hansard*, 18 May 2017, p. 41.

8 Mr Vines, RCA, *Committee Hansard*, 18 May 2017, p. 41.

9 Mr Goulburn, PCA, *Committee Hansard*, 7 June 2017, p. 49.

10 Mr Goulburn, PCA, *Committee Hansard*, 7 June 2017, p. 49.

> **Mrs Shonk:** It is really low. My brother was told up on the Gold Coast that he had a tropical disease even after having [Magnetic Resonance Imaging (MRI)]. When he brought the scan down to Sydney, I looked at it and went, 'That's a brain tumour.' The knowledge with GPs is very limited. My brother-in-law got sent off to the ear, nose and throat specialist. They kind of think about a brain tumour as the absolute last resort, which is kind of unfortunate because time is of the essence. I think it is incredibly poor.
>
> **Mr Shonk:** They do not come across it enough to know what to do.
>
> **Ms Ferguson:** When Leanne first presented herself to the doctor, they did not even take her blood pressure. They just gave her a doctor's certificate—gave her two in case her headache had not cleared up by the next day. In the scheme of things for GBM, a few days is not going to make the difference, but for other cancers, where people are waiting for many months to get a diagnosis, it is almost criminal.[11]

5.9 Professor Terrance Johns of the Brain Cancer Discovery Collaborative (BCDC) remarked:

> That is the problem with a rare disease. It is not only that it is rare and so a lot of the GPs would not necessarily see it very often. The other thing is that patients die so quickly and so they are not continually visiting GPs. A GP might see one patient every five years but then that patient is dead, and so there is no follow-up; there is no corporate memory there. I think that is part of the problem.[12]

5.10 Professor Phyllis Butow, President of the Clinical Oncology Society of Australia (COSA), similarly discussed the need for improved detection and diagnosis of LSR cancers, stating:

> This inquiry will hear a lot about laboratory research, as it should, but we could also do a lot to improve cure rates by simply identifying cancers earlier and treating them more efficiently. To do this we need to understand the blockages in our health system that prevent those things occurring. Rare cancers are particularly at risk of being discovered late because their symptoms are often vague, patients do not know when or how to report them, and GPs are often not very familiar with rare cancers or their symptoms and send patients off in different directions to get different sorts of investigations, because they are not expecting a rare cancer. Patients often say to us that they have been reporting symptoms for some time before they are diagnosed, and they find it difficult to know where to go for expertise and they find it difficult to be reassured that they are on a tried and true pathway for care.[13]

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11 Mrs Margaret Shonk, Mr Evan Shonk and Ms Linda Ferguson, *Committee Hansard*, 18 May 2017, p. 9.

12 Professor Terrance Johns, Director, Brain Cancer Discovery Collaborative, *Committee Hansard*, 18 May 2017, p. 20.

13 Professor Phyllis Butow, President, Clinical Oncology Society of Australia (COSA), *Committee Hansard*, 18 May 2017, p. 30.

5.11 To address low awareness amongst GPs and improve detection and diagnosis, COSA proposed a number of recommendations:

> To improve this, we think there are a number of strategies that might help, requiring health services research. For example, in England and Denmark they have achieved a lot by getting health services to really focus on where the blockages are by setting targets for time to diagnosis after presentation of symptoms and time to treatment after diagnosis, and making the report of achievements against those targets public. This has worked to reduce time frames and get people to care more quickly, but we need to understand what appropriate targets are in the Australian context and how we can accurately measure those time frames. Another opportunity is to implement optimal care pathways, which have been developed by Cancer Council Victoria and endorsed by the National Cancer Expert Reference Group, NCERG. These describe key steps in a cancer patient's journey and the optimal care the patient should receive at each of those steps. We know there is variability in different jurisdictions in the pathway that patients follow, and if we were able to really enforce or encourage uptake of the OCPs we are likely to improve care significantly and reduce some of the disparities that Karen has been discussing. We need to develop implementation strategies to overcome the barriers to implementing those care pathways in different jurisdictions.
>
> We think that a demonstration project of rapid referral clinics may be helpful in this space. For example, we might take a set of symptoms such as abdominal symptoms, which are often the site where rare cancers occur, and have a one-point referral system, where GPs can refer patients with those sorts of symptoms to a clinic personed by GPs who have a particular interest in cancer and who would make sure the possibilities of a cancer are ruled out for those patients, with triaging out to specialists, if that is required. Those GPs would have a very well developed network of specialists to refer out to.[14]

5.12 Cancer Australia informed the committee about the work it does to increase awareness amongst GPs by providing 'evidence-based information, resources and data across the cancer care continuum—so, across a range of cancer types, which obviously does include low survival and low incidence cancers'.[15] Cancer Australia explained:

> We use a range of channels and platforms to present this information and to raise awareness. The Cancer Australia website would be the main one, but also through media releases, media interviews, and through various social media platforms.
>
> If I could give you a couple of examples of our work in raising awareness, with particular relevance to low survival cancers. In our work in Ovarian Cancer Awareness Month in February this year, we developed a range of

14 Professor Butow, COSA, *Committee Hansard*, 18 May 2017, p. 30.

15 Dr Alison Butt, Senior Scientific Officer, Cancer Australia, *Committee Hansard*, 29 August 2017, p. 22 (corrected in correspondence dated 29 August 2017).

> resources for consumers and GPs, with a particular focus on symptom awareness—I think, Senator, you alluded to the challenges that sometimes symptoms can be quite nondescript and fairly common—so there's an important piece of work around raising awareness in the community about what the symptoms are. But also, particularly in the ovarian cancer space, raising awareness for GPs on the importance of the assessment of family history, and also appropriate referrals for ovarian cancer patients. This information was delivered through our Cancer Australia website and through social media channels. The campaign resulted in a 10-fold increase in traffic to the website. So the message is, hopefully, getting out there.
>
> Another example in another low survival cancer is in lung cancer. We have done some work in this space. We developed a video animation, What Your Cough Is Telling You, again working in that important space of raising awareness of symptoms and encouraging members of the public to be aware of what to look out for in lung cancer, and the importance of early investigation of lung cancer symptoms. There are also links on the website to risk factors, to understanding diagnosis and treatment and also for finding support. Again, with this campaign in lung cancer we have seen significant increases in traffic to the website to access this information.[16]

5.13 Cancer Australia also outlined work it undertakes more directly with medical professionals. For example, Cancer Australia convenes 'an intercollegiate advisory group' comprising representatives of the medical colleges and consumers, and which meets twice each year. Through the advisory group and:

> also through a similar mechanism, which is a high-level research and data advisory group, which also meets twice a year, we're able to bring together people who are both working at the coalface and also are policy-makers and health planners and also experts in cancer.
>
> We also work directly with a number of agencies that have mechanisms for accessing GP offices directly. For example, we place messages on the television screens in the GP clinic, and there are a number of point-of-care mechanisms also. Cancer Australia doesn't develop all of them by any means, but there are point-of-care mechanisms whereby the general practitioner in his clinic is able to access relevant information related to patient care.
>
> At Cancer Australia we seek to promote and widely disseminate information that may be of relevance at the community level and at the health professional level. We have the Supporting People With Cancer Grant Initiative, where we work with local communities. We fund them to potentially raise awareness or to provide supportive care to their communities.[17]

16 Dr Butt, Cancer Australia, *Committee Hansard*, 29 August 2017, p. 22.

17 Adjunct Associate Professor Christine Giles, Executive Director, Cancer Australia, *Committee Hansard*, 29 August 2017, p. 23.

5.14 By way of international comparison, the committee heard from The Brain Tumour Charity (TBTC) in the United Kingdom (UK) about its HeadSmart campaign:

> HeadSmart is a campaign that we launched with the University of Nottingham and the Royal College of Paediatrics and Child Health. It's an information campaign…It's about giving parents, carers and also GPs more information about the signs and symptoms of brain tumour in the paediatric population. It's split into three different groups: under-five year-olds, five- to 11-year-olds and 12- to 18-year-olds, as the symptoms can be different. But because the symptoms are very common, it's actually a combination of the symptoms that are the trigger for the referral pathway which was developed by the University of Nottingham under our funding in 2011.[18]

5.15 The HeadSmart campaign has reduced the delay in diagnosis for children with brain cancer from 14.4 to 6.5 weeks;[19] which is still higher than the five week detection period in the US and Poland.[20] Dr David Jenkinson, Chief Scientific Officer of TBTC explained why the UK's detection rates have reduced so dramatically:

> The information given to the carers and parents—often through schools or through nurseries and places like that—is what is really driving the diagnosis. What we are finding, though, is that the teenage group—the 12 to 18s—aren't really getting as good an outcome as the other groups. So the current delay for the 12 to 18 group is 10.3 weeks, whereas with babies it's 4.1 weeks. Obviously, some work needs to be done in that space, which is why the campaign was relaunched with different animations and different graphics as well, hopefully to appeal more to that teenage audience.[21]

5.16 Dr Jenkinson elaborated on how the campaign engages with GPs, while noting that attendance by GPs at 'healthcare professional sessions or days' 'would be less than one per cent':

> A lot of the HeadSmart campaigning is done by a number of volunteer advocates—often people who have been through the situation themselves and have benefited from the HeadSmart campaign. They have found it on our website and then actually gone to their GP with the information to hand. Or there are those who would have benefited had they found it. They are often the best advocates for us to go out there and work with the healthcare professionals, the schools and places like that. We understand that GPs are very busy and that, therefore, another leaflet may not be the best way to educate them.[22]

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18 Dr David Jenkinson, Chief Scientific Officer, The Brain Tumour Charity (TBTC), *Committee Hansard*, 29 August 2017, p. 34.

19 TBTC, *Home*, https://www.thebraintumourcharity.org/ (accessed 19 October 2017).

20 Dr David Jenkinson, Chief Scientific Officer, TBTC, *Committee Hansard*, 29 August 2017, p. 37.

21 Dr Jenkinson, TBTC, *Committee Hansard*, 29 August 2017, pp 36–37.

22 Dr Jenkinson, TBTC, *Committee Hansard*, 29 August 2017, p. 37.

Committee view

5.17 There is no doubt that early detection of cancer significantly improves outcomes and survival rates for patients. Significant improvements have been made to the survival rates for cancers such as breast, prostate and colon as a result of widespread public education campaigns and the availability of tests to aide early diagnosis. The public visibility of these cancers, together with the number of Australians affected and survivors who become advocates, mean that these cancers then tend to attract charitable and philanthropic support, and the majority of funding for research. These cancers are also more likely to be front of mind when a patient presents to their GP.

5.18 As evidence to this inquiry has demonstrated, the rarity of LSR cancers means that GPs infrequently encounter them and this, coupled with often vague symptoms, means that these cancers are not detected and diagnosed quickly. It is obvious to the committee, therefore, that awareness amongst the public and GPs must be improved so that patients seek medical attention and GPs contemplate LSR cancers as a cause sooner than they do currently.

5.19 The committee is impressed by the HeadSmart campaign and the in-roads it has made in reducing the time taken to detect and diagnose brain cancer in babies and young children in the UK. In addition to the valuable work Cancer Australia is already undertaking in this space, the committee is of the view that the Australian government should do more to raise awareness about LSR cancers among the public. The committee recommends that the Australian government develops and implements an education and awareness campaign based on the UK HeadSmart model to inform the public about LSR cancers and their symptoms, with a view to reducing the time taken to detect and diagnose these cancers.

Recommendation 9

5.20 The committee recommends that the Australian government undertakes communication activities targeted at the public with the objective of reducing the amount of time taken to detect and diagnose low survival rate cancers.

5.21 The committee also urges the federal, state and territory governments to consider the proposals made by COSA, and the role that optimal care pathways (OCPs) and rapid referral clinics could play in improving detection and diagnosis of LSR cancers.

5.22 In order to maintain their registration, doctors in Australia are required to undertake ongoing education and professional development, recognition that '[t]he practice of medicine is a constantly evolving field' and so that doctors 'maintain and further develop their knowledge and expertise'.[23] Certain elements of this ongoing training are compulsory (for example cardio-pulmonary resuscitation (CPR)) while others allow 'general practitioners (GPs) to self-identify priority areas of general practice learning needs in accordance with their personal, patients and community

23 Royal Australian College of General Practitioners (RACGP), *Education and professional development*, https://www.racgp.org.au/education/ (accessed 6 November 2017).

needs'.[24] Continuing professional development (CPD) offered by the Royal Australian College of General Practitioners (RACGP), for example, includes courses in vaccination, sexually transmitted infections (STIs), skin cancer, addiction, clinical emergency management, and managing complex pain.

5.23 The committee considers that detection and diagnosis of LSR cancers could form part of this CPD. The committee appreciates that the rarity of LSR cancers means they infrequently present to GPs; however, GPs play a vital role as often the first point of medical contact for a patient with an undiagnosed LSR cancer and it is essential that GPs are sufficiently skilled to identify a LSR cancer as a possible diagnosis early.

5.24 The committee therefore recommends that the Australian government works in collaboration with the medical profession via the RACGP and Australian Medical Association to improve awareness of LSR cancers amongst GPs, including through CPD.

Recommendation 10

5.25 The committee recommends that the Australian government works in collaboration with the Royal Australian College of General Practitioners and the Australian Medical Association to improve awareness of low survival rate cancers amongst general practitioners, including through continuing professional development.

Data

5.26 Data collection and population level information about cancer in Australia impacts on research undertaken into LSR cancers. The committee heard that data collections on LSR cancers in Australia are not as good as they could be, and received a number of suggestions about improvements that could be made in this respect.

5.27 Cancer is a notifiable disease in Australia, such that all state and territory registers are statutorily required to disclose information about cancer to the Australian Institute of Health and Welfare (AIHW).[25] This data is compiled in the Australian Cancer Database:

> …which is a database of all new incident cases of malignant cancers since 1982. It is all cancers not including non-melanoma skin cancer. So we already have detailed data on all new cases of cancer. That covers the number of people who have the cancers. We are also able to bring in information on the deaths from those cancers and can do extensive analysis, including survival analysis, for people with various types of cancer. So there is detailed data there. If there is further information that is required,

24 RACGP, *Planning learning and need (PLAN)*, https://www.racgp.org.au/education/qicpd-program/gps/planning-learning-and-need-(plan)/ (accessed 6 November 2017).

25 Dr Lynelle Moon, Group Head, Health Group, Australian Institute of Health and Welfare (AIHW), *Committee Hansard*, 8 June 2017, p. 14.

> that could always be looked at to see if that could be included in some form.[26]

5.28 However, the committee heard that the Australian Cancer Database only contains data within 'a defined scope', and consequently, data required by a researcher may not be available through this database.[27] For example, the AIHW noted that it has data available 'on non-malignant tumours of the brain (and other parts of the central nervous system) for those diagnosed at any age' but only from Victoria, Queensland, Western Australia and Tasmania, and the Australian Paediatric Cancer Registry—a national cancer registry that specialises in data on cancer in children—contains 'diagnosis data from all jurisdictions, but only for those ages under 15 at the time of diagnosis'.[28]

5.29 Mrs Tricia Berman of the Brain Tumour Alliance Australia (BTAA) opined that Australia 'cannot afford' this approach anymore, noting that 'countries such as the US, Canada and the UK register all [brain tumours], so that is helping, in terms of analysing that data as a researcher, to see what options are available for future treatments'.[29] Further, Mr Philip Steel of BTAA stated that even though it is known that there are 1600 malignant brain tumours recorded in Australia per year, 'we do not really have any idea about how many benign brain tumours there would be, and there is really no way to gather that information'.[30]

5.30 The CSIRO noted that much of the data collected by the AIHW had, until now, been administrative in nature, which required 'researchers to infer clinical utility from the data'.[31] However, the CSIRO considered that the current capturing of clinical data in the Electronic Medical Record and Electronic Health Record means 'that more clinical data is being captured, which if made available, would greatly increase the ability of Australia's medical research community'.[32]

5.31 The CSIRO explained the significance of such data:

> Registries are an important part of Australia's health data landscape. At a state level, health jurisdictions are required to maintain various registries for public health, such as state based cancer registries. In addition, various clinical groups have developed disease specific registries, such as the trauma registry or prostate registry. In the case of mandated registries these

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26 Dr Moon, AIHW, *Committee Hansard*, 8 June 2017, p. 14.

27 Dr Moon, AIHW, *Committee Hansard*, 8 June 2017, p. 16. For information about the requests for data AIHW received from 1 April 2016 to 31 March 2017, see: AIHW, answers to questions on notice, 8 June 2017 and 14 June 2017, (received 3 July 2017), pp 9–10.

28 AIHW, answers to questions on notice, 8 June 2017 and 14 June 2017, (received 3 July 2017), p. 2.

29 Mrs Tricia Berman, Secretary, Brain Tumour Alliance Australia (BTAA), *Committee Hansard*, 8 June 2017, p. 40.

30 Mr Philip Steel, Vice-Chair, BTAA, *Committee Hansard*, 8 June 2017, p. 40.

31 CSIRO, *Submission 204*, p. 10.

32 CSIRO, *Submission 204*, p. 10.

> typically contain a minimum data set and it is a legal requirement to submit this information. In the case of clinical registries, these are typically more detailed but are not mandated and will not capture all cases in Australia.
>
> The linking of data from different data collections to these registries can add significant value. In the case of the cancer registries, the linking of treatment and outcome data provides a more useful set of data for clinical research.[33]

5.32 Professor Eslick argued that people with LSR cancers need to be asked about their lifestyle and that this information must be recorded, as the cause of many cancers, such as pancreatic cancer, is not understood and evidence is needed in order to prevent and treat them:

> Until you identify what causes them, you cannot prevent them and you certainly cannot treat them.…You get information, but you need to be conducting large, prospective studies on people and asking them nitty-gritty questions about: 'What do you think caused your cancer? What has your work been like? What do you eat on a daily basis?' I believe that the majority, probably 98 per cent of cancers, are due to environmental factors, and the remainder are probably due to genetic factors. Some of those environmental factors may switch genes on and off. I think, primarily, unless you can identify these factors, we are sitting in a position where these gentlemen are correct: in 100 years, survival rates for these low-survival cancers have not changed. It is a disgrace. As a researcher, you get a bit shirty when you see all this funding going to breast cancer and colon cancer and other cancers that now have really good survival rates. You think, 'What about the rest?' I think it is time for a change.[34]

5.33 The Cancer Council Australia (CCA) and COSA raised concerns about the accessibility of research data due to articles being 'hidden behind paywalls' as well as 'delayed release [of research data] by long embargo periods'.[35]

5.34 However, the committee was also told that 'there can be a significant administrative burden in the data sharing'.[36] The Cancer Council Victoria (CCV) stated that:

> …what researchers are wanting to do is prioritise those high-value collaborations with institutions that have the capability and capacity to do that. I think there is undoubtedly that appetite. We see in our organisation, and I am sure in Karen's as well, the existence of collaborative institutions coming together with combined research applications to our organisation,

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33 CSIRO, *Submission 204*, p. 10.

34 Professor Eslick, University of Sydney, *Committee Hansard*, 18 May 2017, p. 59.

35 Cancer Council Australia (CCA) and the Clinical Oncology Society of Australia (COSA), *Submission* 137, p. 18.

36 Mr Todd Harper, Chief Executive Officer, Cancer Council Victoria (CCV), *Committee Hansard*, 18 May 2017, p. 36.

> and I am sure others, including [the National Health and Medical Research Council (NHMRC)] as well. I think the appetite is certainly there.[37]

5.35 In light of the difficulties with sharing research data, the CCA and COSA suggested that the Australian government could 'show leadership' by:

> …ensuring that all federal government departments and agencies, as well as cancer research centers [sic], and universities, that fund cancer research are required to adopt and implement open access policies that require knowledge to be openly licensed and freely-available without restrictions or embargoes.[38]

A national biobank

5.36 A biobank is a facility that collects and stores 'various clinical samples, such as blood and tissue from consenting patients for use in medical research'.[39] Biobanks are 'widely recognised as valuable resources for biomedical research' and can improve 'the prevention, diagnosis, treatment and ongoing management of diseases, including cancer'.[40] A range of submitters and witnesses therefore advocated for a national biobank, particularly for brain cancer.

5.37 The Queensland Brain Institute (QBI) explained the importance of cancerous tissue in oncology research:

> **Senator BUSHBY:** Coming back to the tumour tissue, you talk about how valuable it is and about keeping it for research purposes. We also heard earlier that there have fairly recently, I think, been full DNA profiles on tumours.
>
> **Prof. Richards:** Yes.
>
> **Senator BUSHBY:** If you do a full DNA profile of a tumour, is that all the information you need, or are there still advantages in keeping the tissue for other purposes? Just take us through that.
>
> **Dr Bunt:** You want as much tissue as possible which is not necessary for the standard care. Whatever the pathologist does not need is really a source of important information. There are different kinds of tissue preservation methods. We have the pathological tissue, in paraffin, which you can use for looking at the morphology of cells. Indeed, recently people have done a lot of profiling of the DNA, which has changed our whole view about tumours that we thought were just one tumour type; they are actually two or sometimes three different tumour types, or just one but representing differently. We also—and you see that in a lot of big laboratories around the world—want viable tissue, tissue which is still alive.
>
> **Prof. Richards:** Removed from the brain.

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37 Mr Harper, CCV, *Committee Hansard*, 18 May 2017, p. 36.

38 CCA and COSA, *Submission* 137, p. 18.

39 Brain Cancer Biobanking Australia, *Submission 119*, p. 1.

40 Brain Cancer Biobanking Australia, *Submission 119*, p. 1.

> **Dr Bunt:** Yes, because we can use it for xenografting models. That is when you take the tumour and transplant it to a mouse so you can use it for either basic research on understanding how this tumour behaves or drug testing—preferably, in the long term, maybe even models where you can test drugs for a patient on a mouse model with the same tumour. If the patient then has a recurrence, we know what drugs might help. So there are multiple levels there.
>
> But what you see is that the groups that really changed the landscape in our understanding of brain tumours are big groups, and they are collaborating. You need a lot of material from different tumours to really make a difference. Because they are so different, you need at least hundreds of the tumours to really find what they have in common and what makes them become the tumour they are. So that is very important, and you see that countries that have a longstanding culture of archiving and preserving this kind of material now have an advantage, because they have this material ready to go and a lot of information about the outcome for the patient.[41]

5.38 Professor Linda Richards of the QBI explained that brain cancer researchers require both biological and non-biological data:

> We need research that is done by physicists and also mathematicians who are applying algorithms to try to understand how tumours are able to progress and invade the tissue around them'.[42]

5.39 The QBI therefore recommended 'the establishment of a central brain tumour tissue bank' which would provide 'timely access to the tissue needed to develop tumour models'.[43]

5.40 The Cure Brain Cancer Foundation (CBCF) also supported 'national bio-banking and registry linkages', stating that '[s]tate governments are creating impressive data linkages within their states that have the potential to transform research and care' and that:

> The Australian Government is well placed to facilitate the integration of these resources through initiatives, such as the [Coalition of Australian Governments (COAG)] National Cancer Work Plan, so that the national capacity is greater than its parts and to create a truly international competitive research environment with the highest levels of patient care.[44]

5.41 Dr Bryan Day, Team Head, Translational Brain Cancer Research Laboratory at the QIMR Berghofer Medical Research Institute (QIMR Berghofer) and Professor

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41 Dr Jens Bunt, Research Fellow and Team Leader, NFI Research Lines, Brain Development and Disorders Laboratory and Professor Linda Richards, Deputy Director, Research, Queensland Brain Institute (QBI), The University of Queensland (UQ), *Committee Hansard*, 6 June 2017, pp 16–17.

42 Professor Richards, QBI, UQ, *Committee Hansard*, 6 June 2017, p. 19.

43 QBI, *Submission 133*, p. 1.

44 Cure Brain Cancer Foundation (CBCF), *Submission 139*, p. 11.

David Walker described current collection of brain tumour tissue as 'ad hoc'.[45] This is because there are a number of complexities around the collection of human tissue, including the way it is used and stored, issues of ethics and consent, and other patient information gathered.

5.42 Dr Jens Bunt of the QBI explained, in relation to the collection of tissue:

> …there is a lot of tissue which is lost in certain steps, because we have a lot of different hospitals, both private and public, a lot of different pathologists and a lot of different neurosurgeons and because there isn't the awareness that we can use this for basic research. Sometimes it is lost because it is not stored in the right way or the pathologist releases the additional material a little bit too late for us. In our case, because we really want to xenograft it, there is a time limit. We would like it straightaway from the surgeon—within 15 minutes into a mouse.[46]

5.43 Related to the collection of tissue, Professor Michael Buckland and Professor Manuel Graeber discussed neuropathology, in the context of diagnosis of and research into brain tumours. Professor Buckland remarked that:

> Brain Cancer Biobanking Australia…is trying to coordinate brain cancer tissue banks across the country to create a single large virtual biobank to get the sorts of numbers we need for proper studies. I do note that the National Research Infrastructure Roadmap which was recently produced by the federal government did indicate that networked biobanking was a research priority for the government.
>
> I would also like to emphasise the role—the often forgotten role—of pathology and pathological diagnosis in the treatment of these tumours…In many cases, the role of the pathologist is often overlooked. I think, particularly with the government funding models, the role of the pathologist is not supported. I would point out that many of the tests we are now required to do to comply with the latest WHO classification of brain tumours are not Medicare rebatable, so either we have to absorb the costs, the referring doctors absorb the costs or the patients have to pay out of their pockets. In Sydney, many of the large departments will absorb those costs, so we will charge back to the referring hospital. However, I am concerned that in rural and disadvantaged areas there is not that sort of money, so patients are asked to pay and they baulk, and so in fact their diagnosis may not be adequate.
>
> I would put it to you that for any decent treatment you need to know what you are dealing with, and that is the role of the pathologist. Just the other week a large multi-institutional study from the United States was published on the pathological diagnosis of brain tumours. They examined 1,500 brain

45 Dr Bryan Day, Team Head, Translational Brain Cancer Research Laboratory, QIMR Berghofer and Professor David Walker, private capacity, *Committee Hansard*, 6 June 2017, pp 44 and 47, respectively.

46 Dr Bunt, QBI, UQ, *Committee Hansard*, 6 June 2017, p. 17.

> tumours and identified a serious error or misdiagnosis rate of close to five per cent when tumours were diagnosed at a local institution.[47]

5.44 Professor Graeber was similarly strident is his support for neuropathology and emphasised its vitally important role in cancer research:

> People have to have training in neurology and psychiatry—in the brain sciences—in addition to what they do in pathology. You cannot become a properly trained, if you apply international standards, neuropathologist easily except when you make special efforts—like [Professor Michael Buckland]. He keeps travelling and attending international courses. I commend his effort to raise that little flag of the neuropath department. I strongly support that. It is the best thing you can do for brain tumour research and also neuroimaging dementia research in this country. We need proper neuropathology. There are so few hands that look at the brain's hardware…[48]

5.45 Professor Richards of the QBI discussed time delays arising from ethics approval processes, highlighting that '[h]uman ethics is obviously crucially important' but also that:

> I think every tumour patient would want their tumour tissue which is being removed to be used for research purposes. I think that it would be more beneficial to have an opt-out process whereby the patient, if they decided they did not want to have their tissue used for research, would opt out rather than having to opt in, because that is just an extra step of consent that has to go through.
>
> In general, I would say the human ethics is a very, very long process to get approved at the moment. We have the ability to perhaps share with the groups in Europe or in the US, but we would have to de-identify that information. But the ethics of trying to get the ability to even share the de-identified data is very complex, especially at an international level, let alone at a national level. I am not kidding. It can take a year, 18 months, to get one ethics approval at the moment.
>
> **Senator BURSTON:** Could it be part of the consent form for an operation?
>
> **Prof. Richards:** It should be. It really should be. But here, again, we need the buy-in of the clinicians. We desperately need the full buy-in of the clinicians. We have had some supportive clinicians in Brisbane, who made it opt out rather than opt in, and that helped a lot.[49]

5.46 The committee heard that tissue collection cannot occur in isolation, and that information about each patient from whom a tissue sample is collected is essential. Dr Nicola Waddell, Group Leader, Medical Genomics Group at the QIMR Berghofer emphasised that in order:

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47 Professor Michael Buckland, private capacity, *Committee Hansard*, 18 May 2017, p. 57.

48 Professor Manuel Graeber, Barnet-Cropper Chair of Brain Tumour Research, Brain and Mind Centre, University of Sydney, *Committee Hansard*, 18 May 2017, p. 62.

49 Professor Richards, QBI, *Committee Hansard*, 6 June 2017, p. 17.

> …for tissue banking to work, you need to enrich the samples with clinical information. You need to be able to continually follow up the patients, see how they have progressed, and find out what treatment they received and how well they did, because that will inform the samples and the research that is being done on the samples too.[50]

5.47 Wesley Medical Research told the committee that '[w]ithout data, the samples are worth nothing' and that there must be relationships and networks between clinicians, pathologists and researchers so that researchers are:

> …able to go back to the clinician and know when they have had extra testing done—or where the sample has come back and it is a different type of tumour, or they have got a recurrence, the surgeons will ring me and say: 'This patient's coming back in next week. Can you collect?'[51]

5.48 Some submitters and witnesses also discussed logistical, regulatory and cost implications. The CCA and COSA discussed the current fragmentation of biobanks in Australia, describing the sector as:

> …poorly regulated and lags well behind many other countries. Specifically, a current lack of biobank oversight means that the numbers of biobanks that currently exist in Australia, how most of these biobanks operate, and whether they are effectively supporting Australian research by performing at internationally-accepted standards, is not known.[52]

5.49 The QBI similarly described the fragmented nature of Australian biobanks and their differing objectives:

> Already there are multiple tissue banks currently in Australia with different goals and different ways—what kind of material they have and do—so the start is already there, but you have to have local nodes. It would be good when there is just one consensus, both from the researchers and the clinicians, about a concept, so everybody is aware that a clinician cannot say, 'I didn't know that I could provide this tumour,' because it is a standard concept within the clinical environment.[53]

5.50 The QBI and Wesley Medical Research commented on the costs of establishing and maintaining a biobank. Professor Richards explained:

> There is no doubt that a national tissue bank would really help a lot. It will be expensive. Obviously, you need a person there at midnight or whenever the surgical procedure is going on. You literally need somebody there holding the tube while the neurosurgeon removes the tumour and then bringing it back to the bank, processing it and making sure it gets to us as

50 Dr Waddell, QIMR Berghofer, *Committee Hansard*, 6 June 2017, p. 44.

51 Ms Emma Raymond, Theme Leader, Cancer, Wesley Medical Research, *Committee Hansard*, 6 June 2017, p. 31.

52 CCA and COSA, *Submission* 137, p. 18.

53 Dr Bunt, QBI, UQ, *Committee Hansard*, 6 June 2017, p. 18.

> fast as possible so we can then put it into culture or put it into a mouse or whatever. It is not easy. It is complicated to set up a tissue bank.[54]

5.51 Ms Emma Raymond, Theme Leader, Cancer, Wesley Medical Research went on to discuss the financial cost associated with implementing a standardised procedure to collect tissue samples and data from all patients, stating that:

> If the money was not an issue and we did it blanket across every type of tumour, or all the rare types of tumours, and things like that, I think that it would provide a resource for researchers that could be amazing long-term. It is just that every time I have seen someone try to do that, they will get funding for one year or two years or five years, and at the end of it, they have not had enough time to then provide those samples to the researchers, or they have to shut the doors and then what do they do with the samples?
>
> There needs to be a look at, if we are going to do something to the level, a commitment for 20, 30 years at least with the infrastructure built in. You need to have buy-in from the public and private sectors, and that is where it gets difficult. So in the private sector, I can physically go into theatre and stand there and collect the sample, but in the public sector it will not work that way.[55]

5.52 Ms Raymond also spoke to accessing the Brain Bank at the University of Queensland (UQ) and the cost of storing brain tissue samples:

> …the problem is the samples that they have stored [at UQ] are half-brains from motor neuron disease, Parkinson's disease and things like that. To store their samples would cost us approximately $50,000 a year just in electricity. If there were a large resource, it would be great to bring in the little ones like that and provide them to researchers. There is one case over there where four members of the same family all have different types of dementia. Those sorts of samples would be so useful to researchers, but like I said, the actual money involved to bring all those samples across would be a lot.[56]

5.53 Professor Richards suggested that tissue collection and participation in research by clinicians and doctors could be improved by making 'a Medicare rebate contingent upon them providing the tissue':[57]

> …we need an increased awareness of the importance of research in the clinical setting. Hospitals should be made aware of how important it is to have research trained doctors leading their clinical groups. Obviously we need doctors that also focus only on patient care, but the heads of departments, for example, should be trained in research so that they can make sure that that department also contributes to the research effort to cure that disease, no matter what disease it is, not just treat the patient. That is of

54 Professor Richards, QBI and UQ, *Committee Hansard*, 6 June 2017, p. 18.

55 Ms Raymond, Wesley Medical Research, *Committee Hansard*, 6 June 2017, pp 31-32.

56 Ms Raymond, Wesley Medical Research, *Committee Hansard*, 6 June 2017, p. 34.

57 Professor Richards, QBI and UQ, *Committee Hansard*, 6 June 2017, p. 18.

> the utmost importance—I do not want to undermine that at all—but we should be in the process of preventing disease, preventing these tumours from ever happening, and we need to understand why they occur in order for that to happen. It is trying to involve our hospitals somehow. I was not kidding when I said maybe you need to look at the Medicare rebate and whether or not you actually tie that to the hospital, embedding research in that setting.[58]

Committee view

5.54 The committee notes that the Australian government is undertaking some initiatives with respect to data collection. For example, through Cancer Australia, the government:

> …is undertaking an initiative which aims to strengthen national data capacity through the collection, transfer, collation and the reporting of standardised national data on stage, treatment and recurrence (STaR) for all cancers.[59]

5.55 Importantly, the initiative 'is being undertaken in collaboration with relevant Australian Government departments and agencies, and state and territory governments and their population-based cancer registries'; and, according to the government, 'will address the lack of national data on the severity of cancer at diagnosis, which treatments are applied, and the recurrence of cancer after treatment'.[60]

5.56 The committee welcomes this important initiative and urges the Australian government to implement it as a priority, given how important clinical and population level data are to medical research. The committee reiterates the importance of Australian cancer data collections being complete and, aided by technological improvements in both data collection, management and analysis, the committee recommends that the Australian Cancer Database is expanded to capture all cancers, including benign tumours of the brain and other parts of the central nervous system.

5.57 In doing so, and acknowledging consultation already underway with federal departments and agencies as well as state and territory governments, the committee also recommends that the Australian government consults with medical researchers to identify what data (for example, clinical and lifestyle) data must be included so that the Australian Cancer Database is a valuable and useful resource to them.

5.58 The committee also recognises that expanding the data set collected will require the consent and cooperation of patients and clinicians. The Australian government must collaborate with its state and territory counterparts to address

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58 Professor Richards, QBI and UQ, *Committee Hansard*, 6 June 2017, p. 19.

59 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. 13.

60 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. 13.

current barriers to data collection, and consider ways in which data collection can be mandated, standardised and streamlined across Australia, in both public and private health settings.

Recommendation 11

5.59 The committee recommends that the Australian government, in collaboration with state and territory governments:

- **considers expanding the Australian Cancer Database to capture all cancers, including benign tumours of the brain and other parts of the central nervous system;**
- **in so doing, consults with medical researchers to identify what clinical and lifestyle data might be included in order to benefit oncology research; and**
- **addresses current barriers to data collection and considers ways in which data collection can be improved across Australia, in both public and private health settings.**

5.60 The committee welcomes the acknowledgement of networked biobanks as a priority area in the 2016 National Research Infrastructure Roadmap.[61] The Roadmap states that:

> Biobanks are enablers across a range of medical, agricultural and biodiversity research. Integrating existing tissue and environmental biobanks into collaborative networks linked to the research community, ensuring the ability to collect, store and analyse high quality useful research data will provide significant improvement in research effectiveness.
>
> Linking established biobanks into a national network of central tissue repositories will turn an under-utilised product into a more valuable research resource. Under a national system for collecting and biobanking human tissue samples, standards for data gathering and sample curation would assist in the sharing of materials and would foster collaborations. Inclusion of genomics, proteomics and metabolomics data with health, lifestyle and clinical data, will magnify our ability to develop new diagnostics and therapies.
>
> While the necessary institutional processes are in place in the network of natural history museums, herbaria and seedbanks, medical biobanking is fragmented. Australia would also benefit from a population biobank. A population biobank has unique value for population genomics and research into the causes, prevention and treatment of disease. Other countries have well established population biobanks that provide infrastructure for public

61 Australian Government, *2016 National Research Infrastructure Roadmap*, February 2017, https://docs.education.gov.au/system/files/doc/other/ed16-0269_national_research_infrastructure_roadmap_report_internals_acc.pdf (accessed 7 November 2017), p. 72.

> health research…We should explore building on existing capabilities to move towards a national biobank network.[62]

5.61 The committee fully endorses this position and urges the Australian government to give serious consideration to implementing a national networked medical and population biobank that includes tumour samples and relevant clinical and lifestyle data associated with each tumour sample.

Recommendation 12

5.62 The committee recommends that the Australian government gives serious consideration to implementing a national network medical and population biobank that includes tumour samples and relevant clinical and lifestyle data associated with each tumour sample.

Genomic medicine and biomarkers

5.63 Advances in genomic medicine and molecular biology, particularly the identification of biomarkers, are paving the way for 'personalised medicine' and immunotherapy.

5.64 Genomics is the study of the genome; genomic medicine is the medical discipline that uses and applies genomic information to a clinical setting, such as managing a patient's condition or disease, and informing decisions about their care. In cancer genomic medicine, genetic testing may be able to identify the type of cancer, the heritable risk for a cancer, or a targeted treatment of a cancer.[63]

5.65 A biomarker is a naturally occurring molecule found in blood, other body fluid or body tissue that can be a sign of an abnormal process or of a condition or disease. A biomarker may also be used to determine how well the body responds to a particular treatment.[64]

5.66 The Garvan Institute of Medical Research/Kinghorn Cancer Centre/Garvan Research Foundation (the Garvan Institute) explained the significance of genomics to personalised medicine, and the positive impact this form of treatment has on increasing survival rates for LSR cancers:

> The genome is the complete set of genetic information we inherit from our parents, and which determines every aspect of health and susceptibility to disease. Genomic research has given us a new understanding of the interplay within the genes, throughout our whole genetic landscape.
>
> …

62 Australian Government, *2016 National Research Infrastructure Roadmap*, February 2017, p. 71.

63 Australian Genomics Health Alliance, *About genomics*, https://www.australiangenomics.org.au/for-participants/about-genomics/ (accessed 7 November 2017).

64 National Cancer Institute, *NCI Dictionary of Cancer Terms: biomarker*, https://www.cancer.gov/publications/dictionaries/cancer-terms?cdrid=45618 (accessed 7 November 2017).

> Precision or personalised medicine is the future for all cancer treatment, but will have its greatest impact for 'rare', high-mortality cancers. Genomics is essential to precision medicine. More funding is needed for clinical research that brings the potential of genomic medicine to the challenge of 'rare' and lethal cancers.[65]

5.67 The benefits of genomic research for advances in ovarian cancer was outlined by Ovarian Cancer Australia:

> …as we have progressed in genomics, we know that ovarian cancer is not just one disease; it is a group of different types of cancer, each with different cellular appearances and each with different molecular characteristics and different trajectories. New evidence, for example, has revealed that 50 per cent of ovarian cancer comes, in fact, from the fallopian tube and then spreads to the ovaries.[66]

5.68 Professor David Thomas, Head of Cancer Research at the Garvan Institute, discussed the work of he and his colleagues at the Kinghorn Cancer Centre who have developed a Genomic Cancer Medicine Program (GCMP) that 'focuses on "rare" cancers'[67] with the goal of improving cancer outcomes for people with these diseases. The program:

> …brings together researchers and clinicians to translate research findings into the clinic. The program utilises the sequencing capacity of the Garvan Institute of Medical Research to identify more effective treatments for cancer patients, as well as to understand and exploit heritable cancer risk.[68]

5.69 The GCMP's Molecular Screening and Therapeutics (MoST) study 'offers, within the research context, molecular profiling of tumours for patients with "rare" cancers and links this to relevant experimental and standard treatments':

> MoST squarely addresses the challenges of engaging individuals with less common cancers in clinical research, taking advantage of the principles of precision medicine. Eligibility for participation in clinical trials available as part of MoST is completely independent of the 150-year old classifications that arbitrarily divide cancers according to where they arise in the body. Once a cancer has spread, its site of origin is less important for patients than understanding what makes the cancer 'tick'. MoST trials personalise experimental treatment based on an individual's unique personal and cancer genetic profile, and in so doing neutralise the disadvantage of 'rarity'. MoST offers a new kind of clinical trial of treatments targeted to the genomics of patients with high-mortality cancer and unmet clinical need.

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65 Garvan Institute of Medical Research/ The Kinghorn Cancer Centre/ The Garvan Research Foundation (Garvan Institute), *Submission 34*, p. 1.

66 Ms Jane Hill, Chief Executive Officer, Ovarian Cancer Australia, *Committee Hansard*, 4 August 2017, p. 2.

67 The Garvan Institute defines rare and less common cancers as those 'affecting up to 12 in 100,000 people', which 'account for 23.7% of cancers diagnosed, and 38.5% of cancer deaths': Garvan Institute, *Submission 34*, p. 2.

68 Garvan Institute, *Submission 34*, p. 1.

…

> Until recently, clinical trials were generally used to test a new treatment, with some patients getting the new drug and the others getting an existing drug or placebo. The MoST protocol tests multiple treatments at the same time and all participating patients receive a treatment. The advent of personalised medicine means that treatment is guided by the genetic make-up of the patient and their illness.
>
> First, all patients, and their tumours where possible, are genomically screened to see if they are suitable for a trial and if there are biomarkers that can guide the treatments that can be trialled. These 'signal-seeking' trials are looking to see if a treatment will work, or work more effectively than another treatment. The MoST protocol looks to understand how targeted therapies work and find new biomarkers that can predict which patients will benefit from these treatments.
>
> After screening, patients will be offered one of three options:
>
> 1. MoST clinical trials, including immunotherapies
>
> 2. Clinical trials outside MoST that use molecular eligibility criteria
>
> 3. Other biomarker-guided treatments outside MoST.
>
> All participants, including those with no 'actionable' biomarkers, will be informed of the results of the screening of their tumour tissue through their own doctors.[69]

5.70 The MoST protocol 'is also conducting clinical studies to test novel immunotherapy drugs in patients with high-mortality cancers' through two separate studies, although, it is noted that while 'immunotherapies are proving to be effective in many cancer types, they do not work in all patients':

> MoST researchers are looking to find biomarkers that can predict which patients will benefit from specific treatments targeting the immune system and to better understand how immunotherapies work to fight cancer. With this knowledge, the team aims to develop a more precise approach that tailors treatment with immunotherapy to individual patients based on the characteristics of their immune system and its interactions with tumour cells.
>
> The immunotherapy trials will allow us to understand how these immune biomarkers influence the anti-tumour response and help develop a precision immunotherapy approach where treatment can be personalised.[70]

5.71 The Garvan Institute established the GCMP in collaboration with the NHMRC Clinical Trials Centre and with the support of the New South Wales government.[71] The NHMRC has also:

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69 Garvan Institute, *Submission 34*, pp 5–6.

70 Garvan Institute, *Submission 34*, p. 6.

71 Professor David Thomas, Director, The Kinghorn Cancer Centre; Head, Cancer Research Division, Garvan Institute, *Committee Hansard*, 8 June 2017, p. 31.

> …committed $27.5 million from the [Medical Research Endowment Account] to support the International Cancer Genome Consortium (ICGC) between 2009 and 2014.
>
> The ICGC is a confederation of members (mostly key funding agencies in major countries) that agreed to work in a coordinated and collaborative manner to characterise a minimum of 500 unique cases for 50 different cancer types or subtypes that are of the highest clinical and societal importance across the world. The aim was to obtain a comprehensive description of the full range of genetic events associated with these tumour types and make the data available to the entire research community as rapidly as possible, and with minimal restrictions, to accelerate research into the causes, diagnosis and control of cancer.
>
> The $27.5 million grant was awarded to Professor Sean Grimmond (Institute for Molecular Biosciences, University of Queensland). This funding supported two large Australian-based projects to characterise ovarian and pancreatic cancers. The ICGC has now evolved into ICGC medicine (ICGCmed) that will link genomics data to clinical information, health and response to therapies.

5.72 In speaking to this funding grant, Dr Elizabeth Johnson of the Victorian Comprehensive Cancer Centre (VCCC) informed the committee that it 'allowed Australia to go to the forefront of pancreatic cancer research in particular'.[72]

5.73 Indeed, Professor Johnson noted that the VCCC chair, Professor Sean Grimmond:

> …is now leading genomic approaches particularly in pancreatic cancers. Australia is now a world leader on that, with that allocation of funding for a specific purpose for a limited amount of time having seeded something very significant that has now put us at the forefront of research in pancreatic cancer genomics. So there is precedent for it to happen that way.[73]

5.74 The NHMRC has also funded the Genomics Revolution in Health Care program, in 2015 providing:

> $25 million in funding for a Targeted Call for Research (TCR) into Preparing Australia for the Genomics Revolution in Health Care (for funding commencing in 2016). The aim of this targeted call was to support research that will provide evidence and information that could be used to help prepare Australian policy and practices for implementation of genomic information into health care. NHMRC sought to fund a single, multidisciplinary, nationally focussed grant through this TCR.
>
> The funded application supports a national alliance of clinicians, researchers, health economists and policymakers to evaluate the case for

72 Dr Elizabeth Johnson, Program Manager, Victorian Comprehensive Cancer Centre (VCCC), *Committee Hansard*, 7 June 2017, p. 40.

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

> clinical genomics across inherited disease and cancer, and to determine how best to deliver this to the patient and to train a capable workforce.[74]

5.75 Cancer Australia has also contributed to genomic research in Australia, in 2013 establishing and funding:

> …the *Genomic Cancer Clinical Trial Initiative* to provide [National Cancer Cooperative Trials Groups] with expert advice and technical services relating to the collaborative development of genomics-based clinical trials protocols. From 2013 to the present, this initiative has led to the development of 17 new concepts for genomics-based clinical trial protocols across multiple cancer types, including a multicentre, randomised study specifically focussed on new treatment approaches in rare cancers.[75]

5.76 Cancer Australia remarked that:

> Recent advances in genomics have increased our understanding of cancer at the molecular level, leading to new approaches to diagnosis and treatment. Genetic sequencing technology has enabled cancers to be re-classified based on a specific tumour mutation (or mutations) rather than the site of origin of the cancer. This has led to the development of genomics-based clinical trials that test a therapy or combination of therapies targeted to the mutation across multiple cancer types, and can provide important insight into the effectiveness of targeted treatment interventions. Genomics-based clinical trials present opportunities for patients with low incidence cancer types to join…larger clinical trials based on the genomic profile of their cancer, rather than its site of origin.[76]

5.77 Despite government funding for genomics research via the NHMRC and Cancer Australia, Professor Stephen Fox, Director of Pathology at the Peter MacCallum Cancer Centre informed the committee that:

> Most international countries of any ilk have large, stratified medicines programs independently funded outside basic science routes, which is the NHMRC or even the [Medical Research Future Fund (MRFF)]. They have large precision medicine programs in the US, as well as institutional ones. In the UK you have got Genomics England, a genomics centre in Scotland and there is even a genomics centre in Wales. In Australia I think we are a little bit behind there. And there is a genomics centre in Kuwait as well, I believe.[77]

5.78 Professor Fox also informed the committee that, from a testing point of view, the regulatory process in Australia has not caught up with genetic advances in

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74 National Health and Medical Research Council (NHMRC), *Submission 87*, p. 4.

75 Cancer Australia, *Submission 129*, p. 7.

76 Cancer Australia, *Submission 129*, p. 7.

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

understanding cancers, noting that Australia is 'way behind international benchmarking'.[78] Professor Fox elaborated:

> The amount of genomic genetic testing available on the [Medicare Benefits Schedule (MBS)] is absolutely minimal, and what there is, is usually tied to a particular drug. So we have nothing in our armamentarium to provide diagnostic tools. We get no reimbursement for that. Indeed, should we try to make a proposal through the [Medical Services Advisory Committee (MSAC)] process for some of the tools that we require, we can't fulfil the requirements because the evidence base is so small. So for example, when you want to do a generic platform and apply it to multiple tumour types—because you are looking for a genetic change, as opposed to a particular tissue stream—you are not able to do that whatsoever, which is very disappointing.[79]

5.79 Recognising that Australia lags behind in some aspects of genomics, the CCA and COSA advocated for 'new, longer-term and more flexible funding grants…to enable the development and maintenance of equipment, technologies and other large-scale research infrastructure such as *biobanks* and genomics services'.[80]

5.80 Roche explained that '[b]y looking beyond the "site" of a cancer to its molecular biology and understanding the true complexity of the disease, we can find solutions that work for both common and rarer cancers'.[81] The CBCF observed that '[o]ver the past few years we have begun to see the importance of biomarkers in cancer control', and advocated for using biomarkers 'whenever possible to provide another layer of important information for both clinician and patient…[which] has the potential to result in better targeted treatment and better health outcomes'.[82]

5.81 However, the committee heard that there are barriers to genetic testing and identification of biomarkers in Australian LSR cancer patients. For example, NSW Oncology Group (NSWOG) Neuro-oncology noted that there are issues of equity that currently affect individuals with particular sorts of cancers:

> At present detailed characterisation of individual patient tumours is available only in a research setting. While common genetic alterations such as mutations in the IDH gene are routinely tested as part of pathology, further analysis is not made available for the vast majority of patients – clearly limiting the ability of the treating team to potentially tailor treatment to that is best for the patient. The correlation of this is that this may alter survival rates adversely.[83]

78 Professor Fox, Peter MacCallum Cancer Centre, *Committee Hansard*, 4 August 2017, p. 49.

79 Professor Fox, Peter MacCallum Cancer Centre, *Committee Hansard*, 4 August 2017, p. 49.

80 CCA and COSA, *Submission* 137, p. 18 (emphasis in original).

81 Roche, *Submission 124*, p. 6.

82 CBCF, *Submission 139*, p. 11.

83 NSW Oncology Group (NSWOG) Neuro-oncology, *Submission 123*, p. 4.

5.82 Ms Linda Ferguson discussed how the lack of government rebates through the MBS prevented her wife from undergoing tests that Ms Ferguson believes would have assisted other patients who shared the same biomarker(s):

> I do recall Leanne was offered a particular blood test when we first moved to the Gosford healthcare system. I cannot recall exactly what this test was for, whether it was looking for genetic markers or methylation status of the tumour—I just cannot remember—but I recall we were told it would not be refunded through Medicare and that we would be out of pocket about $350 for doing it. Leanne asked the doctor how would her treatment be done differently depending on the results of the test, and we were told that there would be no change to her treatment regardless of the results. This made us think, well, why would we pay $350 for a test that is not going to help her—so we did not do that one. In retrospect, with the benefit of hindsight and with a better understanding of the circumstances in which these doctors are working, I now believe this test was not offered to help Leanne but was offered instead to help future patients. It was a way of giving the doctors additional information—an extra variable to add to the mix to help them make decisions about future patients who might share the same characteristics as Leanne.
>
> So I guess we were being asked to pay for information that was essentially adding to what is known about brain cancer. We were being asked to pay for this ourselves because no-one else was paying for it. I do not begrudge doctors learning from patients—indeed, with rare cancers I believe we must learn something from each and every patient—but for that cost to be borne by the patient or their family when brain cancer already places the heaviest financial burden on households and has the highest per person lifetime economic cost, it is simply wrong. If there is a blood test or a suite of blood tests that that could provide some of the missing jigsaw puzzle pieces, then surely we owe it to our loved ones, if they are willing, to do these tests and for them not to have to pay for them. At the very least, why couldn't the cost of these tests be covered by Medicare?[84]

5.83 The CBCF also advocated for the reimbursement of biomarker testing.[85]

5.84 Bristol-Myers Squibb (BMS) identified the increasing use of biomarkers in oncology as a 'positive step in improving patient health outcomes', but stated that 'the requirements to fulfil both the [Pharmaceutical Benefits Advisory Committee (PBAC)] and MSAC processes add complexity and evaluation time':[86]

> Clinical trial design for cancer medicines is providing real challenges to the reimbursement process, it is exceedingly difficult for the newer cancer agents to prove cost effectiveness against the older cytotoxic agents. This is primarily due to one of the key reimbursement criteria being the requirement to demonstrate cost effectiveness against the comparator,

84 Ms Linda Ferguson, *Committee Hansard*, 18 May 2017, p. 2.

85 CBCF, *Submission 139*, p. 11.

86 Bristol-Meyers Squibb (BMS), *Submission 289*, p. 3.

> defined as the treatment that is most likely to be replaced in clinical practice.
>
> However with the rapid emergence of new cancer medicines, the treatment landscape is rapidly evolving and as such, the appropriate comparator for the purposes of evaluating cost-effectiveness may not be known at the time the trial is designed for the assessment of safety and efficacy. This poses a problem because it is quite likely – and most often the case – that the appropriate 'main comparator' nominated within a reimbursement submission is not the comparator(s) of the Phase III clinical trials.
>
> In this case, the therapeutic efficacy and safety of the new medicine relative to the appropriate comparator has to be estimated indirectly from clinical trials with a common third comparator. This is less methodologically rigorous than the direct comparison method. In fact, the PBAC has a low acceptance of using indirect comparisons to substantiate claims of clinical superiority and cost effectiveness. [87]

5.85 Professor Andrew Wilson, the Chair of PBAC agreed that 'one of the challenges' is 'what's the right comparison?':

> It's challenging in that it's tempting for companies to say, 'This drug works better for this smaller group of patients,' and then they can get a better price for the drug, so then they don't go and examine these other patients whom it may benefit. It's challenging in that those same markers may be just predictors of a tumour which is going to behave well or behave badly anyway, so they may be a prognostic marker: if you've got that, your tumour's going to do better or your tumour's going to do less well. And then we give you this drug and, lo and behold, you seem to do better compared to the others, but actually it's related to the biology of the tumour itself.[88]

5.86 In contrast to the optimism about genomics and biomarkers expressed by other submitters and witnesses, Professor Wilson also stated:

> If you believe the hype at the moment, you would think we were there, that we could characterise tumours on the basis of some form of genomic mapping or some sorts of markers, and we'd be able to choose just the perfect drug for you. Unfortunately, while there are many promising aspects of this, we are still quite a substantive way away from where this is likely to be widespread.[89]

Immunotherapy and personalised medicine

5.87 Immunotherapy refers to a treatment:

> …that uses certain parts of a person's immune system…to fight cancer. Immunotherapies are thought to work by slowing the growth and spread of

87 BMS, *Submission 289*, p. 3.

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

89 Professor Wilson, PBAC, *Committee Hansard*, 29 August 2017, p.18.

> cancer cells, and by helping the immune system destroy existing cancer cells.[90]

5.88 According to the NHMRC, personalised medicine applies knowledge about genetics to predict disease development, influence decisions about lifestyle choices and/or tailor treatment to an individual. As a result, personalised medicine is expected to:

> …result in better disease prevention and more accurate diagnosis of disease. Personalised medicine could also use knowledge of the way specific genes work with medicines to tailor more effective treatment of disease for each individual.[91]

5.89 During the course of the inquiry, both the immunotherapy and personalised medicine were identified as important areas of development in the treatment of LSR cancers, and a source of hope for LSR cancer patients and their families.

5.90 Ms Susan Pitt, a consumer advocate, stated that '[w]e already have surgery, chemotherapy and radiation, but the big brave new area is immunotherapy…That is a big area of hope for patients'.[92] Professor Buckland described 'the new wave of immunotherapy for melanoma' as a 'great example' and 'a very exciting new area of oncology'.[93] The CBCF stated:

> Immunotherapy in other diseases has become quite revolutionary. Diseases like melanoma, which typically had a poor prognosis, are actually seeing great improvements in survival. Melanoma is a solid tumour, just as brain cancer is a solid tumour. We understand that there are significant differences, but we are looking at a number of activities to look at immunotherapy of all different types in brain cancer. Some of the results are promising. It is not quite as exciting as melanoma yet—we have not really cracked that—but there is definitely evidence to suggest that it is an area worth considering.[94]

5.91 Merck Sharp & Dohme (Australia) (MSD) described immunotherapy as '[o]ne of the most promising innovations in cancer treatment' and explained why it is focussing its research on immunotherapies:

> Initially, when the immunotherapy mechanism of action—this concept that your immune system is used to fight against the tumour—came about, I think what quickly became apparent was that you could use this treatment,

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90 Cancer Australia, *Immunotherapy*, https://canceraustralia.gov.au/affected-cancer/treatment/immunotherapy (accessed 7 November 2017).

91 NHMRC, *Personalised medicine and genetics*, November 2013, https://www.nhmrc.gov.au/_files_nhmrc/publications/attachments/g004_personalised_medicine_genetics_150622.pdf (accessed 7 November 2017), p. 1.

92 Ms Susan Pitt, *Committee Hansard*, 8 June 217, p. 4.

93 Professor Buckland, private capacity, *Committee Hansard*, 18 May 2017, p. 63.

94 Ms Michelle Stewart, Head of Research Strategy, CBCF, *Committee Hansard*, 6 June 2017, p. 27.

> rather than its being a targeted treatment in a specific tumour, that all of a sudden this mechanism of action had applicability across multiple tumours, which offers a real opportunity and low survival rate in rare cancers, frankly. So, we have embarked on what we call a tsunami of work, really, which is trying to test or trial this drug in multiple tumours really at the same time…[95]

5.92 Like BMS (see paragraph 5.83), MSD raised the difficulties of getting an immunotherapy listed on the Pharmaceutical Benefits Scheme (PBS). Both MSD and the CBCF noted that the US Food and Drug Administration (FDA) has taken a different approach to assessing immunotherapies[96] and clinical trial protocols for LSR cancers:

> We funded an international project with 160 researchers coming together to talk about this based in Arizona State University. All the researchers were from all over the world and from top institutions. The protocol was written and submitted to the FDA, and we thought it was quite ambitious. The FDA came back and said, 'Be more ambitious. This is the future of drug development. We would like to see treatments developed around the disease, not by the pharmaceutical company.' So this would act as a platform. Rather than companies like Pfizer or Roche running their own trials, this would be done by a number of pharmaceutical companies at the same time. Also, rather than going from a phase 2 trial to a phase 3 trial, which could take six years, this would compress the phase 2 and phase 3 trials, reducing it down to a couple of years. So you can see it would reduce significant cost, reduce significant time and, also, act as an incentive for biopharmaceutical companies to get involved in the area.[97]

5.93 In an October 2017 report commissioned by MSD, Deloitte Access Economics made the following recommendations with respect to improving awareness, availability and affordability of immunotherapies:

- to improve awareness:
  - change the language to one of survivorship and immunotherapy as a potentially transformative alternative for many patients, where appropriate;
  - ensure patients have access to reputable and evidence-based information, setting out what immunotherapies are available in Australia for whom, and how to access them as they are emerging through the pipeline, including information on biomarker testing where appropriate;
  - remove sectoral silos and develop partnerships between research, industry and academia, with patients in the middle, to help ensure patients and clinicians can navigate information channels effectively; and

95 Ms Zoe Armstrong, Clinical Research Director, Merck Sharp & Dohme Australia (MSD), *Committee Hansard*, 6 June 2017, p. 53.

96 Ms Armstrong, MSD, *Committee Hansard*, 6 June 2017, pp. 52-55.

97 Ms Stewart, CBCF, *Committee Hansard*, 6 June 2017, p. 25.

- provide further support to survivors who face financial constraints, such as counselling services and return to work programs.[98]

- to improve availability:
  - systemic change similar to what has recently been demonstrated by the [United States' Food and Drug Administration], adopting a tumour agnostic approach that recognises molecular level treatment;
  - increased investment and coordination in availability of biomarker and screening tests, to better target therapies towards biomarkers that are likely to respond;
  - faster implementation of the new mechanisms available since the [Therapeutic Goods Administration (TGA)] Review; and
  - greater awareness among oncologists of the TGA's provisions for special access.[99]
- to improve affordability:
  - capacity constraints in PBAC processes need to be overcome to ensure that listing of new medicines is not delayed as increasingly more fill the pipeline, since the speed of listing is critically important and cancer is already the slowest therapeutic area to be reimbursed;
  - reimbursement decisions in PBAC need to link with TGA tumour agnostic assessments across a range of therapeutic outcomes, with serious consideration of new models for funding immunotherapies into the future;
  - recognising the substantial cost of innovative biological molecules, affordability considerations should include life-saving and compassionate access to trials; and
  - the entirety of benefits from newer medicines need to be valued including not just health system, longevity and quality of life impacts, but also productivity and other impacts on patients, carers and society. Data should be captured in trials.[100]

5.94 The committee also heard that the regulatory framework in Australia differs to that in the European Union (EU) and United States (US), which provide greater flexibility for basket studies that use biomarkers, and can have positive results for people with LSR cancers:

> Innovative trial designs are being explored to support access to treatments for rare diseases, where it is not feasible to conduct randomised trials. Studies known as "basket studies" look at a patient group with a mix of tumour types that have common biomarkers, rather than conducting studies

98 Deloitte Access Economics (Deloitte), *The New Wave of Immunotherapy Cancer Medicines – The Untapped Potential for Australians*, October 2017, p. 60.

99 Deloitte, *The New Wave of Immunotherapy Cancer Medicines –The Untapped Potential for Australians*, October 2017, p. 60.

100 Deloitte, *The New Wave of Immunotherapy Cancer Medicines –The Untapped Potential for Australians*, October 2017, p. 61.

> in each tumour. However, such studies are not currently accepted as an evidence base by the Therapeutic Goods Administration, the [PBAC] nor the [MSAC], although they are more acceptable by EU and [US] regulators. There needs to be further discussion on the role of these types of basket studies when making decisions on access to treatments for rare diseases, especially as there is some excellent research currently being conducted in Australia using these types of trial designs.[101]

Committee view

5.95 Advances in genomics, molecular biology, personalised medicine and immunotherapy offer hope and the possibility of innovative and effective treatments for LSR cancer patients. Australia is well served by so many passionate and committed medical researchers in these fields, who work tirelessly and without widespread recognition, and who have to endure the vagaries of uncertain funding streams. Supporting their efforts is vital if improvements are to be made to the survival rates for LSR cancers.

5.96 The committee hopes that the recent changes to the NHRMC's funding model will see genomics and other research into personalised medicine and immunotherapies funded over periods that enable researchers to substantively progress their work. The committee expects that its recommendations in relation to data and a national biobank will also assist medical researchers and support further advances.

5.97 However, the committee shares the concerns of NSWOG Neuro-oncology, the Peter MacCallum Cancer Centre and others that Australia is lagging behind comparable countries in its support for genomics and provision of routine genetic testing of LSR cancer patients. The Australian government should ensure ongoing funding for genomic research, through organisations such as the Kinghorn Cancer Centre. The government should also consider reimbursing LSR cancer patients for genetic testing, via the MBS, both to contribute to scientific understanding of these cancers and also to assist in the identification of personalised treatment for LSR cancer patients in the future.

Recommendation 13

5.98 The committee recommends that the Australian government ensures ongoing funding for genomic research into low survival rate cancers.

Recommendation 14

5.99 The committee recommends that the Australian government implements any recommendation from the Medical Services Advisory Committee to list genetic tests for low survival rate cancer patients on the Medicare Benefits Schedule so that these tests are routinely available to these patients and reimbursed.

5.100 The committee acknowledges the government's implementation of some of the recommendations arising from the medicines and medical devices review

101 Medicines Australia, *Submission 141*, p. 12.

(MMDR), in relation to the TGA's approval processes, and its commitment to implement others (see chapter 2). The committee welcomes the reduction in regulatory barriers for the supply of certain unapproved therapeutic goods and expedited review of 'vital and life-saving prescription medicines'. The committee urges the TGA to implement the other recommendations, particularly the provisional approval pathway that will provide earlier access to new medicines without a full dossier of clinical data but where there are potentially substantial benefits to Australian patients.

5.101 Further and with respect to the use of clinical trials based on biomarker rather than tumour location, and having an immunotherapy approved for use and listed on the PBS, the committee believes it is essential that the TGA and PBAC (re-)examine their assessment processes and the appropriateness of those processes for innovative treatments for LSR cancers. The committee finds it unacceptable for a "one size fits all" approach to be applied to the assessment of innovative treatments, such as immunotherapies, for LSR cancers when it is clear that the existing approaches are ill-suited to these treatments and no improvements in survival rates for these cancers have been made. Put simply, if it is acceptable for European and American regulators to adopt more flexible and innovative approaches to assessing immunotherapies—including approval or acceptance of novel clinical trial protocols—the committee sees no reason why, pending a (re-)examination of TGA and PBAC assessment processes, more flexible and innovative approaches should not be adopted in Australia.

Recommendation 15

5.102 The committee recommends that the Therapeutic Goods Administration, if necessary following the medicines and medical devices review, and the Pharmaceutical Benefits Advisory Committee:

- **(re-)examine their assessment processes and the appropriateness of those processes for innovative treatments for low survival rate (LSR) cancers, such as immunotherapies; and**
- **pending that examination, consider adopting more flexible and innovative approaches to approving innovative treatments for LSR cancers and assessing them for listing on the Pharmaceutical Benefits Scheme.**

Access to medicines

5.103 The committee heard that in some instances, there are medicines available that may assist in treating LSR cancers, but that these drugs are approved for use in Australia for a different indication or are not approved and available for use in Australia at all. Equity of access and the availability of medicines via the PBS was also discussed during the course of the inquiry.

Repurposing drugs

5.104 The Thoracic Society of Australia and New Zealand argued that there are many drugs already approved for use that may be effective in treating LSR cancers, describing the use of these drugs as 'a low-risk avenue to increase possible cancer therapies':

> This approach takes drug molecules which have already been designed, developed, characterised and tested for safety and efficacy in humans and applies them to a new formulation, method, or target. It is estimated that most safe-approved drugs will possess secondary indications for use in another setting. This will be a time and cost saving endeavour. There are numerous examples for drugs currently in use which were originally developed to treat a different illness.[102]

5.105 A number of other submitters and witnesses also supported the repurposing of drugs approved for other indications as potential treatments for LSR cancers.[103] RCA remarked that '[t]here are many opportunities to repurpose existing drugs from common to rare cancers, but we need evidence and flexibility'.[104]

5.106 Professor Johns explained how the physiology and biochemistry of the brain make drug treatment difficult,[105] and outlined how the BCDC engages with pharmaceutical companies to test drugs used for more common forms of cancer as possible treatments for brain cancer:

> The way that I mostly do it is that they will develop a drug, say, for breast cancer or lung cancer, that we believe might have utility in brain cancer, but they are not interested that because the finances do not make sense as it is rare, so we will work with them to get some of the drug and maybe a little bit of money, and develop the background and do the preliminary experiments in the test tube and animal models to give them the confidence to move forward with that drug in this space. So, it is through partnerships with them. They can come to the groups like the [BCDC] and see that we have the ability to take their drug through all of the tests and evaluations they need to do to be confident to move it forward into brain cancer. That is certainly one thing that we are very focused on and have done in the past, but we still need the basic research to know the companies to approach that have the right drugs that might be effective.[106]

5.107 RCA highlighted research being undertaken at the Garvan Institute:

> …at Garvan, there is a trial being run by Professor David Thomas which looks at analysing the genetic make-up of tumours and then trying to define treatments from existing drugs. There is so much opportunity in this process to repurpose. We have got a whole arsenal of drugs on the shelf here, but we just need to go through—they may have been developed for breast cancer, lung cancer or bowel cancer, but, if we are really clever about it, we can run trials, test them and, we might find…that the drug that was developed for lung cancer is ideally suited…We need to do work in that

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102 Thoracic Society of Australia and New Zealand, *Submission 103*, p. 5 (citations omitted).

103 See, for example, Professor Richards, QBI, UQ, *Committee Hansard*, 6 June 2017, p. 20; RCA, *Submission 50*, p. 12.

104 Mr Vines, RCA, *Committee Hansard*, 18 May 2017, p. 38.

105 Professor Johns, BCDC, *Committee Hansard*, 18 May 2017, p. 24.

106 Professor Johns, BCDC, *Committee Hansard*, 18 May 2017, p. 21.

> area, and David Thomas has set up a trial that, like all research, is hard to fund, but it is an example of what is possible.[107]

5.108 TBTC spoke to the approach in the UK, and noted the role that charity organisations can play in helping pharmaceutical companies repurpose drugs:

> I think that there are differences between a drug that's still on patent, and therefore being driven by a company and their ability to make profit, and one that's off patent. There was a bill that was put to parliament to bring about an easier way of taking those off-patent drugs forward, but that didn't make it through. There are currently discussions around putting that bill forward again.
>
> I think that when we're talking about a drug that's effectively a cancer drug for a different cancer type and moving that into brain or pancreatic, and when that's under patent by a company, then, as charities, we have a role to play in helping the company facilitate that, because the company still has the barrier of the investment versus the return, and we don't have that barrier. So we would like to be able to work more closely with companies and access their drugs to be able to do those trials. There are continuing to be discussions around that. I personally feel that the industry is becoming more open to those approaches. I think there's just some work to do to maybe make them easier still.
>
> The off-patent drugs are a challenge, because this will have to be funded through charitable or not-for-profit organisations. Personally we don't have any problem with a researcher bringing us those sorts of applications. Whether or not they would ever become licensed is the problem, because then the question would be: who would actually submit for the licence application? I think that that's where we need to make some changes to allow that to be an easier thing to do and also to give some indemnity for that person. For example, as a charity, we wouldn't be able to bring a drug to market, because of the potential risks to the charity were that drug to be found at a later stage to be harmful.[108]

5.109 Indeed, internationally, there are other innovative approaches to incentivise pharmaceutical companies to perform clinical trials to repurpose drugs, as the QBI explained:

> Big pharmaceutical companies will not start a clinical drug trial for a rare disease where there are not many, but they will do anything to be able to sell more. Actually in Europe there are a few initiatives where, for instance, if they actively seek to repurpose drugs for rare diseases, they can keep their patent for a couple of months longer or have an advantage over competitors.[109]

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107 Mr Vines, RCA, *Committee Hansard*, 18 May 2017, p. 39.

108 Dr Jenkinson, TBTC, *Committee Hansard*, 29 August 2017, p. 39.

109 Dr Bunt, QBI, UQ, *Committee Hansard*, 6 June 2017, p. 20.

Listing on the Pharmaceutical Benefits Scheme

5.110 Equity of access to medicines for patients with LSR cancers as compared with patients with more common forms of cancer was also the subject of discussion during the inquiry. In particular, submitters and witnesses highlighted that some drugs are available via the PBS for patients with certain cancers, but not for LSR cancer patients, or have been approved and are available for use overseas but not in Australia.

5.111 For example, Ms Ferguson explained that her partner, who suffered from neutropaenia as a result of chemotherapy for brain cancer, was not entitled to the same treatment as those patients with breast cancer and neutropaenia.[110]

5.112 RCA gave an example of a woman with anaplastic lymphoma kinase (ALK) positive cancer for which:

> There is no known diagnosed treatment for this on the PBS, but there is a version of lung cancer that is also caused by that mutation. Through a process of initially paying for the medicine through our crowdfunding service and then, subsequently, through us and her clinician, lobbying the pharmaceutical companies, she is now on a compassionate program for those drugs.[111]

5.113 Ms Marilyn Nelson told the committee:

> What can happen, and has happened to someone I know…is that her doctor did not actually tell her about this drug because he was weighing up the cost of presenting her with something that she could not afford. He chose not to tell her about this drug. The only way she could get it was to pay about $8,000 a month. He did not tell her—she found out about it through other sources. She said, 'I'm going to pay it—we'll mortgage the house, we'll find money somehow.' It is ongoing at $8,000 a month. Eventually Rare Cancers Australia helped her with some crowd funding and then eventually it got on the PBS, but it was months and months of paying thousands of dollars to get access to a drug that is already approved and in use in the [US] and [EU]. It has part of the approval—maybe the TGA approval—in Australia, but it is going through these painfully long processes for getting approval on the Pharmaceutical Benefits Scheme. As patients, we know this drug is there. We know it is being used everywhere, not here. Then we find we can actually get it as long as we are prepared to take out a mortgage on our homes. It is something we face a lot, and we find all this information ourselves...[112]

5.114 Mrs Evangeline Lim, a lung cancer patient, described her 'constant fear that I will run out of treatment options, let alone be offered a cure'.[113] Mrs Lim described herself as lucky that Xalkori, a targeted treatment, is available to her, but also told the

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110 Ms Ferguson, *Committee Hansard*, 18 May 2017, p. 8.

111 Mr Vines, RCA, *Committee Hansard*, 18 May 2017, p. 38.

112 Ms Marilyn Nelson, *Committee Hansard*, 6 June 2017, p. 10.

113 Ms Evangeline Lim, *Committee Hansard*, 6 June 2017, p. 3.

committee that 'America is more advanced as far as pills and treatments go' and that three other treatments have recently been approved in America, but are not available in Australia.[114]

5.115 With regard to the absence of PBS-listed treatments for LSR cancers, RCA stated that:

> It is no small coincidence that government research funding into rare cancers remains disappointingly and disproportionately low, as does the money we spend on treatments for these patients through the Pharmaceutical Benefits Scheme. These two are closely related, as research generates evidence to justify PBS funding, and it is a direct consequence that the drugs are not listed on the PBS; it is a lack of research.[115]

5.116 Mr Vines of RCA continued:

> The PBS requires evidence of cost-effectiveness. I always describe it like this: imagine that the only way you would decide what car you bought was on the basis of fuel economy. The decisions the PBS makes are not entirely but largely driven by improvement in survival for a cancer patient. If the current drug gives you three years and the new drug gives you four years, you have an extra year, so the cost related to that is balanced off. And that is regardless of what the side effects are. There is no measure of the side effects; there is no measure of how many times you are hospitalised or anything like that.
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> …
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> The second part of that is that you have to look at the pharmaceutical industry, and, for a patient population of 30 or 40 in Australia, there are two restrictions: one is, do they have any evidence at all and have they run a trial on that? And secondly, putting in an application to the PBS is a big job. As a charity, we applied to list two drugs last year so we understood the process. Aside from the financial investment, they have a team of people whose job it is to make applications to the PBS. If I were running that team, sitting there, I would say: do I make an application for this drug here, which might be melanoma or breast, which will give me thousands of potential uses, or do I make it for Merkel cell carcinoma, which is going to give me 300? I only have a certain number of hits.
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> So we need to think about how we make that a bit easier...one of the things we have thought about is: can we make it so that they can apply for several at the same time and bundle them up to make that process more efficient?[116]

5.117 The committee also received evidence about the difficulties with respect to the interaction between the PBAC and the MSAC processes.[117] For example, MSD

114 Mrs Lim, *Committee Hansard*, 6 June 2017, p. 6.

115 Mr Vines, RCA, *Committee Hansard*, 18 May 2017, p. 38.

116 Mr Vines, RCA, *Committee Hansard*, 18 May 2017, p. 40.

outlined how a co-dependent submission—'those submissions where they rely on a drug and a test combination'—can delay access to drugs:

> With the co-dependent submissions…the patient would first need to be tested for a particular biomarker and then, provided that the patient has a particular biomarker, then they would qualify for treatment with the drug. That is called co-dependent submissions or co-dependent products in Australia. The challenge we face with those types of products is that we have the test which is funded through a separate committee—MSAC. And then we have the drug that is funded through a separate committee, the PBAC, which we are all familiar with. And the process of integration between the two is problematic.
>
> The process needs to start early, especially on the test site, and that is where it takes almost twice as long as for the drug, because we need to start the process very early on, sometimes when we do not even have some data in order to be able to go through the process. It is the interaction between those two committees. They do not meet at the same time. There are complexities associated with putting forward the health economic arguments. There is an expectation around certain types of evidence which does not happen overseas. That type of information might not necessarily always be available in the clinical studies.[118]

5.118 Professor Fox of the Peter MacCallum Cancer Centre stated that the applications to PBAC and MSAC—which are submitted simultaneously—are 'out of sync', and recommended that the delays in the process could be assisted by aligning the committees, such that they communicate with each other more.[119] Professor Fox also suggested that the PBAC and MSAC be merged into a single committee.[120]

5.119 This recommendation for a single committee was also made by MSD,[121] which noted that 'reimbursement submissions are often co-dependent technology applications, requiring submissions to both the PBAC and MSAC, which can significantly lengthen approval timelines'.[122] In addition to its recommendation for a single committee, MSD also recommended that the Australian government:

- conducts a review of evidentiary expectations for co-dependent applications and benchmarks these to comparable reimbursement authorities overseas
- implements a framework for a managed entry scheme for diagnostics used in co-dependent technologies, similar to what has been in place for

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117 As illustrated in Figure 4, PBAC determines whether drugs should be listed on the Pharmaceutical Benefits Scheme, and Medical Services Advisory Committee which services, devices, consultations or allied services should be listed on the Medicare Benefits Scheme.

118 Mr Carmel Spiteri, Team Leader, Market Access, MSD, *Committee Hansard*, 6 June 2017, p. 53.

119 Professor Fox, Peter MacCallum Cancer Centre, *Committee Hansard*, 4 August 2017, p. 27.

120 Professor Fox, Peter MacCallum Cancer Centre, *Committee Hansard*, 4 August 2017, p. 27.

121 MSD, *Submission 115*, p. 3.

122 MSD, *Submission 115*, p. 3.

> pharmaceuticals since 2011, to enable access to patients whilst more conclusive evidence is being generated.[123]

5.120 Similarly, Medicines Australia advocated for streamlining 'the evaluation and decision making process for co-dependent medicines', on the basis that 'medicines for rare or low survival cancers often rely on the use of a diagnostic to identify the appropriate patient population'.[124]

5.121 Professor Wilson, Chair of the PBAC, emphasised the importance of '[f]it-for-purpose clinical trials that inform [PBAC's] decision-making', and noted that where an international trial takes place, 'we don't understand how they work within the services which are available within Australia', and 'the treatment plans and treatment approaches for some of these tumours may vary within the Australian context'.[125]

5.122 Professor Wilson also stated that, in order to list a drug on the PBS, '[w]e would certainly want to see the evidence from a trial', noting that '[i]f a drug's going to be used and promoted broadly in the community then there needs to be substantive evidence that it works and not just, "You might want to try that"'.[126]

5.123 In response to the discrepancy in neutropaenia treatment for brain and breast cancer, Professor Wilson told the committee:

> The decision about the listing of Filgrastim and the other variations on the same drug were based on the cost-effectiveness. So patients develop [neutropaenia] at different rates, depending on what chemotherapy regimes they happen to be on. There are chemotherapy regimes which have high and low rates of [neutropaenia]; there are ones which have very low rates of [neutropaenia]. The original approval for the drug would have been based on the regimes which caused the higher rates of [neutropaenia] in relation to that. Having said that, we are currently in the process of negotiation around an extension of that, so I can't say any more about it. But we have been approached to look at that more broadly and are currently working on that.[127]

Committee view

5.124 The committee applauds the research of institutions such as the BCDC and the Garvan Institute investigating whether certain drugs already used in the treatment of more common cancers, and even other diseases, might be repurposed for use in the treatment of LSR cancers.

5.125 The committee believes that institutions such as the BCDC and the Garvan Institute should be supported to conduct further research into repurposing existing drugs. Consistent with its other recommendations, the committee recommends that the

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123 MSD, *Submission 115*, p. 3 (citations omitted).

124 Medicines Australia, *Submission 141*, p. 13.

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

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

127 Professor Wilson, PBAC, *Committee Hansard*, 29 August 2017, p.17.

Australian government ensures that funding is available to researchers investigating whether existing drugs may be suitable for treating LSR cancers.

Recommendation 16

5.126 The committee recommends that the Australian government ensures funding is available to researchers investigating whether existing drugs may be suitable for treating low survival rate cancers.

5.127 The committee also notes the approach in Europe where pharmaceutical companies that actively seek to repurpose their drugs for rare conditions are able to extend their patent or have an advantage over competitors. The committee therefore recommends that the Australian government works with industry to consider a mechanism to repurpose drugs appropriate for the Australian context.

Recommendation 17

5.128 The committee recommends that the Australian government works with industry to consider a mechanism to repurpose drugs.

5.129 There may be circumstances in which an existing drug is found to be an effective treatment for LSR cancers, but because it is off-patent or the patient population is so small, it is not financially attractive or clinical evidence is insufficient for a pharmaceutical company to seek TGA approval. The committee is aware that there can be serious implications for clinicians and patients, with respect to adverse reactions, indemnity and insurance, of using drugs 'off-label' and that off-label use must be approached with caution. However, the committee heard from some medical researchers and clinicians, and many patients (or their families) that LSR patients with no other treatment options should be afforded the opportunity to access off-label drugs. Indeed, as Mrs Sandra Woods told the committee: '[i]f you are diagnosed with a fatal illness, you have got nothing to lose. You will die anyway, no matter what you try. Trying is doing something; it is fighting back'.[128]

5.130 The committee cautiously agrees, and recommends that consideration is given to permitting off-label access to drugs for LSR cancer patients without further treatment options, on compassionate grounds.

Recommendation 18

5.131 The committee recommends that the Australian government considers a mechanism to permit access to and properly supervise use of off-label drugs for low survival rate cancer patients without further treatment options, on compassionate grounds.

5.132 The committee is concerned by the apparent inequity of access to some treatments for LSR cancer patients via the PBS. The committee understands that pharmaceutical companies may make financial decisions not to seek PBS listing for medicines to treat rare and LSR diseases, and that the PBAC's evidentiary and cost-effectiveness requirements mean that many drugs for rare and LSR diseases will not

128 Mrs Sandra Woods, *Committee Hansard*, 18 May 2017, p. 13.

obtain PBS listing if sought. However, it is unacceptable that LSR cancer patients should be left without access to treatments which are available to other Australian cancer patients or people in foreign jurisdictions. The committee has already recommended that the TGA, if necessary, and the PBAC (re-)examine their approval and assessment processes for innovative treatments for LSR cancers; the committee makes the same recommendation with respect to the repurposed drugs.

Recommendation 19

5.133 The committee recommends that the Therapeutic Goods Administration and Pharmaceutical Benefits Advisory Committee examine the appropriateness of their approval and assessment processes for existing drugs repurposed for use in low survival rate cancers.

5.134 With respect to co-dependent submissions requiring MSAC approval of a diagnostic test or tool and PBAC assessment of a drug, the committee agrees with the proposals that these processes should be better aligned and streamlined.

5.135 The committee has already recommended that the Australian government considers listing genetic tests for LSR cancer patients on the MBS; where a treatment for LSR cancer is dependent on a genetic or other diagnostic test, the committee recommends that the Australian government considers whether the MSAC and PBAC processes can be streamlined so that assessment and approval is not unduly delayed.

Recommendation 20

5.136 The committee recommends that the Australian government considers whether the Medical Services Advisory Committee and Pharmaceutical Benefits Advisory Committee processes can be streamlined where a diagnostic test and treatment for a low survival rate cancer are co-dependent.

Care and support services for patients and families

5.137 A number of submitters and witnesses expressed their frustration and disappointment about difficulties accessing care and support services, such as care co-ordinators or nurses and welfare payments.

Care and support services

5.138 The committee heard from a number of submitters and witnesses that being diagnosed with a LSR cancer can cause patients to feel isolated and unsupported.[129] QIMR Berghofer suggested that this is a consequence of the rarity of the cancers, such that '[t]hey are not common enough to justify specific support services at all centres', despite the fact that such care is important, regardless of where patients live.[130]

5.139 For example, Mr Tim Eliot recounted the problems that he experienced with respect to receiving information about research and treatment options, including that '[t]echnical documents supplied post-surgery, such as pathology reports, often have

129 See, for example, QIMR Berghofer, *Submission 80*, p. 7; Australia and New Zealand Melanoma Trials Group, *Submission 167*, p. 3; Mrs Karyn Harris, *Submission 185*, p. 2

130 QIMR Berghofer, *Submission 80*, p. 7.

little explanatory or interpretive information beyond what is provided verbally', and that there are '[i]nformation gaps in how to access and best use available care and support services, including Cancer Council; Allied Health; Public/Private cancer care choices'.[131]

5.140 These issues appear to be exacerbated for people living in regional and remote areas. For example, Mrs Suzanne Turpie informed the committee about the lack of support available for her son, a brain cancer patient, once he leaves the metropolitan area in which he receives his treatment:

> We have pretty much no help. As soon as we leave Sydney we are on our own. We see our local GP, who I cannot fault, though she is not a specialist in the field at all. It is only when we go to Sydney every three months that Caleb gets the support and help that he needs. We are desperately crying out for Caleb to be able to see a psychologist right now. After everything that has happened to him, he has terrible nightmares, terrible dreams, and it is impacting his life quite a lot. We cannot get in to see a psychologist in Port Macquarie at all. We are screaming out. We just cannot get into one. So he only gets mental help when we go to Sydney, and that is not good enough. He needs help and he cannot get it.[132]

5.141 Ms Dianne Dunn, who lives 45km from a major regional town and was diagnosed in November 2016 with an inoperable brain tumour, shared a similar experience.[133] Ms Dunn outlined a number of difficulties that she faced with respect to her diagnosis, such as her inability to easily seek a second opinion about her initial cancer diagnosis, and suggested that such difficulties could be addressed by '[p]roviding greater access to those in regional areas to support services – transport to treatment, accessing second opinions'.[134]

5.142 Another issue raised was the difference in support available for patients depending on their cancer. For example, Ms Ruth Churchill stated:

> There is very little support in the community beyond tea and sympathy for those with Atrial Sarcoma. Compared to breast cancer sufferers, we are stumbling about in the dark attempting to find information and support services. My family and I have dedicated many hours over the past four years to researching different scientific based treatment approaches and whom to approach for up to date information. – something that we finally feel we are making some headway with.[135]

5.143 This was also discussed by Ovarian Cancer Australia, which provided comments from women with ovarian cancer in response to a recent survey:

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131 Mr Tim Eliot, *Submission 43*, pp 3–4.

132 Mrs Suzanne Turpie, *Committee Hansard*, 18 May 2017, p. 12.

133 Ms Dianne Dunn, *Submission 189*, p. 1.

134 Ms Dunn, *Submission 189*, p. 2.

135 Ms Ruth Churchill, *Submission 76*, p. 1.

> The first lady writes: 'So much pink support makes you feel like you have the wrong cancer. Breast cancer patients even get free parking at the hospital I went to for chemo. Ovarian patients do not.' The second lady writes: 'At the hospital when I get infusions there are dedicated breast cancer support nurses for those getting chemo for breast cancer. Not for me. I have the wrong cancer.' The third lady writes: 'It feels like ovarian cancer is where breast cancer was 30 years ago. It comes down to funding and research.'[136]

5.144 Indeed, a number of submitters and witnesses raised the issue of care co-ordinators/ nurses, as outlined in the following section.

Care co-ordinators and nurses

5.145 Many people with a LSR cancer or their family members expressed their disappointment about the lack of specialist care co-ordinators or nurses, calling for more of these positions,[137] a sentiment supported by organisations and medical professionals.[138]

5.146 For example, in response to a question about the support services he is receiving, Mr Shonk—who was diagnosed with a grade 3 brain tumour in 2004—stated:

> Virtually zero. There aren't any. The one care nurse that they have in the North Shore hospital is half-funded by Ramsay Health Care; the other half is funded by SNOG—the Sydney Neuro-Oncology Group. For breast cancer—I think I am right—they have about 90 care nurses, and some of those patients have a lumpectomy as opposed to a mastectomy. Brain cancer is so much more insidious; it goes on so much longer and it is so much more debilitating. The inequities are just mind-boggling.[139]

5.147 The potential benefit of specialist care co-ordinators and nurses for LSR cancer patients was outlined by a Lung Cancer Nurse Co-ordinator:

> Our hospital offers a dedicated lung cancer [Multi-Disciplinary Teams (MDTs)] which aims to improve patient care and outcomes through the development of an agreed treatment plan. As a specialised Lung Cancer Nurse Coordinator I am involved in the nursing care of our patients with lung cancer in all treatment areas and am an integral part of the MDT. I am an expert point of contact for our patients, providing both psychosocial and clinical support. My experience after 14 years in this field is that supporting patients with lung cancer to receive coordinated care is not only the best

136 Ms Hill, Ovarian Cancer Australia, *Committee Hansard*, 4 August 2017, p. 2.

137 See, for example, Mrs Lyndal Lean, *Submission 77*, p. 2; Mrs Kathy Farrell, *Submission 154*, p. 1; Mrs Harris, *Submission 185*, p. 2.

138 See, for example, Mark Hughes Foundation, *Submission 113*, p. 2; NSWOG Neuro-oncology, *Submission 123*, p. 5; Professor Walker, *Submission 269*, p. 5.

139 Mr Evan Shonk, *Committee Hansard*, 18 May 2017, p. 12.

> way to care for them but is also greatly appreciated by our patients, their families and carers.[140]

5.148 Mr Khang Chiem expressed his appreciation for the care his partner received from a dedicated neuro-oncology nurse care co-ordinator at St Vincent's Hospital, stating that:

> Without her our journey through the public hospital system would have been chaotic, confusing and demoralising. With her gentle and caring approach, she has guided us from the first operation all the way through to the multiple neurosurgeon appointments, and bridged the gaps between the various departments of the public hospital on our behalf. Any questions we had, she, time and time again, found the answers. Due in large part to the nurse care coordinator, my partner has received the best care we could ask for as a patient in the public health system.[141]

5.149 However, the committee heard that there are only a small number of these nurses available relative to the number of people who suffer from LSR cancers. For example, in 2016, when 12 000 people were diagnosed with lung cancer,[142] the Lung Foundation Australia reported that there were 29 dedicated cancer care co-ordinators/lung cancer nurses in 60 MDTs in Australia.[143] Mrs Sandra Woods noted that '[t]here is one online dedicated NETs nurse for all of Australia where there are over 10,000 known NETs patients'.[144]

5.150 Indeed, the committee heard that it often falls to charities or community organisations to raise funds for specialist care co-ordinators and nurses. For example, the Centre for Community-Driven Research (CCDR), a non-profit organisation with the goal of supporting 'a more patient-driven health sector', established the 'Patient Engagement in Research and Services – with One Nurse' program, which:

> …gives a patient access to a registered nurse (via telephone or video) who can help them access all available local services, understand clinical trials that are available to them, and be a [single], central point of support for as long as the patient needs them.[145]

5.151 The CCDR informed the committee that it had piloted the program in pancreatic cancer over the past 12 months, and is currently testing its transferability in brain and ovarian cancer.[146]

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140 Lung Foundation Australia (LFA), *Submission 89*, Appendix: *Improving outcomes for Australians with lung cancer. A Call to Action*, 2016, p. 10.

141 Mr Khang Chiem, *Submission 110*, p. 1–2.

142 LFA, *Submission 89*, p. 1.

143 LFA, *Submission 89*, Appendix: *Improving outcomes for Australians with lung cancer. A Call to Action*, 2016, p. 10.

144 Mrs Sandra Woods, *Submission 7*, p. 4.

145 Centre for Community-Driven Research (CCDR), *Submission 49*, p. 1.

146 CCDR, *Submission 49*, p. 1.

5.152 Ms Michelle Bradley noted that:

> …the McGrath Foundation has worked hard to raise funds to support Breast Care Nurses who offer a range of support for breast cancer patients. This type of support would greatly assist brain cancer patients to negotiate a complicated and daunting treatment pathway which includes surgery, radiation therapy, chemotherapy, medications (such as dexamethasone) and to explore potential side effects.[147]

5.153 The BTAA noted its financial support for brain cancer nurses/care co-ordinators and other brain tumour allied health professionals, and also supported the calls of patients 'for better access to brain cancer care coordinators', on the basis that 'they play a critical role linking patients with treatments and with clinical trials, as well as assisting them to navigate the medical system following diagnosis'.[148]

5.154 The BTAA suggested that while general cancer care co-ordinators are available across most Australian states and territories these 'are not aware of the specific needs of brain tumour patients' and that:

> Specialised cancer care coordinator nurses create efficiencies in the system by freeing up other specialists and can assist with recruitment to clinical trials. As suggested previously, while there have private and private/public models to provide specialist nurses for cancers such as breast, prostate and some others, we are calling for equitable access for all Australian cancer patients with a poor prognosis.[149]

5.155 Dr Jonathon Parkinson, Chair of the NSWOG Neuro-oncology similarly remarked:

> This is the area in which I think we have the opportunity to make the single, most immediate, impact on survival of brain cancer patients: through care coordinators. Over the last few years most of the dedicated brain cancer care coordinators have given way to more general care coordinators covering a number of cancers, who then become preoccupied, sheerly because of the numbers of other types of cancer sufferers. In fact, I think there are only two dedicated care coordinators in New South Wales. We can look at the model of breast cancer as a cancer where care coordinators have made a great impact on survival. I think the care coordinators are even more important to brain cancer sufferers, because of this impact on the family and the resources consumed.[150]

5.156 The Department of Health (DoH) acknowledged the benefits of specialist cancer care co-ordinators and nurses, but stated that '[i]t's not always viable to have specific tumour nurses for all types of cancer', further stating that:

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147 Ms Michelle Bradley, *Submission 108*, p. 5.

148 BTAA, *Submission 127*, p. 14.

149 BTAA, *Submission 127*, p. 15.

150 Dr Jonathon Parkinson, Chair, NSWOG Neuro-oncology, *Committee Hansard*, 19 May 2017, p. 14.

> There is evidence that cancer care coordinators improve patient experiences. It's a difficult, challenging time, and there are lots of care pathways to navigate. Coordinators can help in that information transfer and stitching things together for people. You'd probably be aware that there are cancer care coordinator positions in jurisdictions across Australia which recognise that need to streamline patient care and help support patients across the journey. For the majority of cases, those coordinators are employed, and sometimes receive specialist training roles, through state and territory governments. They're usually nurses who are experienced in cancer care. Some are tumour specific and many are not. The role of those cancer care coordinators varies according to the area in which they're employed, the tumour types and the complexity of patient care needs. Metropolitan cancer care coordinators are generally based at a single institution, often a cancer hospital big enough to have coordinators for the care of patients with just one tumour type, though that's not always the case. Cancer care coordinators in rural areas tend to have to cover a number of tumour types and are often more community based. The overall shortage of nurses is an issue that the Australian health system is facing. In a workforce shortage situation, you need to balance the need for more general nursing positions against increased numbers of nurses for specific roles like cancer care coordinators.[151]

5.157 The DoH informed the committee that the Australian government 'makes a small contribution' to cancer care nurses 'by funding a certain number of the McGrath Foundation's breast care nurses and a certain number of the Prostate Cancer Foundation of Australia's prostate cancer nurses', which the department acknowledged are, incidentally, cancers with the highest rate of survival.[152]

5.158 The DoH further remarked that while 'those coordinators do help in the survival journey':

> …in the context of the total number of cancer care coordinator positions in Australia, it's a fairly small contribution, and that states and territories, because of their responsibility for public hospitals and cancer centres, are generally the employers of [the nurses].[153]

5.159 In contrast to the situation in Australia, the committee heard that in the UK '[t]here are clinical nurse specialists for high-grade [brain] tumours…that coordinate the care of the individual'.[154] These nurses 'will make sure they are getting access to physio and allied health professional services'.[155]

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151 Ms Alice Creelman, Assistant Secretary, Department of Health (DoH), *Committee Hansard*, 29 August 2017, pp 14–15.

152 Ms Creelman, DoH, *Committee Hansard*, 29 August 2017, p. 15.

153 Ms Creelman, DoH, *Committee Hansard*, 29 August 2017, p. 15.

154 Dr Jenkinson, TBTC, *Committee Hansard*, 29 August 2017, p. 38.

155 Dr Jenkinson, TBTC, *Committee Hansard*, 29 August 2017, p. 38.

5.160 In respect of pancreatic cancer in the UK, the approach is that 'each person should be assigned a clinical nurse specialist once they've received their diagnosis to help them navigate the system' through the National Health Service, although some patients are not assigned a nurse as 'it is a role that is in fairly short supply'.[156]

Financial assistance

5.161 In addition to the absence of support co-ordinating their care, the committee heard about the out-of-pocket expenses facing people with LSR cancers, and the challenges facing patients and their carers trying to access financial support.

5.162 Some submitters discussed the financial impact of an LSR cancer diagnosis and the financial burden of repeated diagnostic tests and treatments. For example, Dr Parkinson remarked that 'the financial impact goes with that…when you think of people being cut down in the prime of their earning lives'.[157] Mrs Margaret Shonk commented:

> Yes, definitely support, and also subsidies of the medication, the MRIs and those sorts of expenses. Usually it is the major wage earner that is hit. You are hit with all these extra expenses. Obviously research is key, but those other things would also help with the suffering that many people face when they have someone in the family with a brain tumour.[158]

5.163 Mrs Turpie stated:

> I am still unable to return to work. I was the main income earner in our family, and there is no possible way that I can return to work. We still have to come to Sydney every three months for the next four years, and that is a massive financial impact on us, with travel costs and accommodation.[159]

5.164 Mrs Turpie has been unable to work since her son's diagnosis with brain cancer, and described the difficulties she encountered accessing a carer's pension:

> When I was filling it out it was very much directed at what I thought was an autistic child, high functioning, along those lines. There was nowhere in the form where I could tick that Caleb had cancer and had neurological problems as a result of the surgery, he was in a wheelchair, he was going to get sicker than what he already was and he was going to require this and that. The questions were was he suicidal, did he get up and walk away from his bed at night, did he need to be restrained, was he at risk of leaving the house? He was not at risk of any of that because he could not walk—he could barely even talk at this stage. I could not tick 'yes' to the boxes that they wanted ticked.

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156 Ms Leanne Reynolds, Head of Research, Pancreatic Cancer UK, *Committee Hansard*, 29 August 2017, p. 38.

157 Dr Parkinson, NSW Oncology Group – Neuro-oncology, *Committee Hansard*, 19 May 2017, p. 18.

158 Mrs Margaret Shonk, *Committee Hansard*, 18 May 2017, p. 8.

159 Mrs Turpie, *Committee Hansard*, 18 May 2017, p. 8.

> A friend of mine heard my plight and she got onto our local MP, after he had been knocked back twice, and at the same time I was trying to get onto a social worker from Centrelink to ask what it was that I needed to do, saying that we needed help here. She said we needed to be ticking the boxes that yes Caleb is suicidal—you need to be making a worst case scenario, otherwise it will not get approved. So I ticked the boxes and at the same time the local MP got involved, he rang the office and lo and behold it was approved that afternoon.
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> …
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> It was ridiculous. I had all the letters from the specialist stating what the diagnosis was, what the outcome was, and what we were looking at happening, but I could not hand any of it over. I had to tick the boxes, but it is hard to tick the boxes when the boxes are not aimed at cancers.[160]

5.165 The difficulty with navigating the Centrelink system was also reflected in evidence from Mrs Tracey Taylor, whose son also has brain cancer:

> Because everything happened so fast, you have to get applications in by due dates and times and the amount of information that they are asking for—yes, some of it is relevant; some of it could be different—and then you are left to phone up to ask these questions. You are on the phone for hours, literally hours, and then you are on hold for hours. Then it goes to a dead end and you have wasted three hours of your day. It is time that you do not have. It is like you need—not a fast track, but some kind of extra assistance to say, 'Okay, this person doesn't have time to be sitting on the phone for hours.'[161]

5.166 Mr Phil Reynolds, whose wife died from brain cancer, described his frustration with navigating government agencies and the time it took to access services:

> In my time caring for my wife the most frustrating task was trying to deal with Centrelink, Medicare, ATO, banks and numerous other institutions. Whilst trying to do the best for Caroline I was having to spend up to two days every week on the phone or waiting for my name to be called at these places and often sent away because another piece of paperwork or information was required.[162]

5.167 The Sydney Neuro-Oncology Group commented that '[n]avigating Centrelink and the [National Disability Insurance Scheme] is impossible', elaborating that:

> Most cannot return to work, and even those on higher incomes often have mortgage and family commitments. The need for constant supervision also impacts on the spouse, children and often elderly parents. Studies have documented the stress in caregivers for this cancer is often higher than the patients themselves, but treatment programs and research rarely extends to

160 Mrs Turpie, *Committee Hansard*, 18 May 2017, p. 13.

161 Mrs Tracey Taylor, *Committee Hansard*, 6 June 2017, p. 8.

162 Mr Phil Reynolds, *Submission 240*, p. 2.

> the unpaid volunteers and the long-term impact on children is unknown. Family and carers face the emotional turmoil of being told their loved one is unlikely to survive and have to confront the daily fear of seizures and the challenges of both cognitive deficits and personality change, all compounded by financial stress.[163]

5.168 Dr Rachel Harris, the daughter of a man with brain cancer, argued that the Disability Support Pension (DSP) and the carer's pension 'need to be streamlined'.[164]

5.169 Indeed, there appears to be limited access to the DSP for people with LSR cancers. Following a simplification of DSP assessments from 1 July 2010, a person who has a terminal illness or profound disability is eligible for fast-tracking to prevent these claimants being 'unnecessarily referred for a Job Capacity Assessment and provide them with financial assistance more quickly'.[165]

5.170 A 'manifest grant of DSP' can be made when a claimant is diagnosed with one or more of the conditions listed in Table 7. There are other conditions, listed on the Department of Social Services website, where a manifest grant of DSP can be made when a claimant is diagnosed with one or more of the conditions; undertakes additional action (such as confirming the stage of disease or establishing the prognosis and/or level of care required); and provides evidence that the claimant 'is clearly qualified for DSP'.[166]

163 Sydney Neuro-Oncology Group, *Submission 130*, p. 3.

164 Dr Rachel Harris, *Submission 229*, p. 3.

165 Department of Social Services (DSS), *Fast-Tracking Disability Support Pension Claims for People With Profound Disability Or Terminal Illness*, 7 November 2014, https://www.dss.gov.au/our-responsibilities/disability-and-carers/benefits-payments/disability-support-pension-dsp-better-and-fairer-assessments/fast-tracking-disability-support-pension-claims-for-people-with-profound-disability-or-terminal-illness (accessed 23 October 2017).

166 DSS, *Fast-Tracking Disability Support Pension Claims for People With Profound Disability Or Terminal Illness*, 7 November 2014.

Table 7: Fast-tracked DSP List 1[167]

Letter	Condition	Manifest Category
A	Amyotrophic Lateral Sclerosis (ALS)	Nursing home level care
	Angelman Syndrome	Nursing home level care
C	Creutzfeldt-Jacob Disease (CJD) - Adult	Nursing home level care
G	Gallbladder cancer	Terminal illness
	Gioblastoma Multiforme (brain tumour)	Terminal illness
L	Lesch-Nyhan Syndrome (LNS)	Nursing home level care
	Liver cancer (primary cancer)	Terminal illness
M	Mantle cell lymphoma (MCL)	Terminal illness
	MPS III (San Filippo Syndrome)	Nursing home level care
	MPS VII (Sly Syndrome)	Nursing home level care
P	Patau Syndrome (Trisomy 13)	Nursing home level care
	Peritoneal Mesothelioma	Terminal illness
	Plural Mesothelioma	Terminal illness
	Prader-Willi Syndrome	Intellectual disability
S	Sjogren-Larsson Syndrome	Intellectual disability
	Small cell cancer of the large intestine	Terminal illness
	Small cell cancer of the ovary	Terminal illness
	Small cell cancer of the prostate	Terminal illness
	Small cell cancer of the uterus	Terminal illness
	Small cell lung cancer	Terminal illness

Committee view

5.171 Evidence before the committee demonstrates the benefits to patients of cancer care co-ordinators or nurses and the support they provide. The availability of such support has resulted in improvements to survival rates for those with some cancers, such as breast or prostate cancer.

5.172 Submitters and witnesses to this inquiry have argued that the benefits to patients with LSR cancers may be even greater, given the complexity of their care and the current lack of co-ordinated care and support.

5.173 The committee does not wish to suggest that the level of care and support provided to those with cancers with higher survival rates, such as breast or prostate, should be diminished, and the committee in no way criticises charities that have raised awareness about and provided support for patients with these cancers. Indeed, the committee applauds the work of organisations such as the McGrath Foundation for the

167 DSS, *Fast-Tracking Disability Support Pension Claims for People With Profound Disability Or Terminal Illness*, 7 November 2014.

incredible work they do and the support they provide. However, it is disappointing that LSR cancer patients do not have access to the same care and support. The absence of specific care and support through specialist cancer care co-ordinators or nurses further exacerbates existing inequalities for LSR cancer patients and hinders improvement in survival rates for these people.

5.174 The committee is particularly concerned that the Australian government, via the DoH, appears to only provide financial support for cancer care co-ordinators for patients with breast and prostate cancer. It should not be left solely to charitable organisations to fund and establish specialist cancer care co-ordinators and nurses for LSR cancers: raising awareness and funding can be difficult for these charities because the cancer they represent is so rare and, tragically, very few patients survive long enough to become advocates. It is also unacceptable that LSR cancer patients should have to rely on charities to receive adequate care and support, given the potentially inconsistent and uncertain flows of charitable and philanthropic funding.

5.175 The Australian government should examine how it allocates funding for cancer care co-ordinators and ensure that LSR cancer patients have access to specialist cancer care co-ordinators and nurses. In doing so, the Australian government should work with its state and territory counterparts to improve access to specialist cancer care co-ordinators or nurses in every state and territory. The committee expects that the provision of this care and support will make tangible improvements in the survival rates for LSR cancer patients.

Recommendation 21

5.176 The committee recommends that the Australian government, in conjunction with its state and territory counterparts, works to improve access to specialist cancer care co-ordinators or nurses for low survival rate cancer patients in every state and territory.

5.177 The financial costs to LSR cancer patients can be large and this can place an immense burden on them and their families. It is concerning that people who are already vulnerable and fighting for their lives are further burdened with loss of income and the financial stress of large medical bills.

5.178 In the first instance, the committee is of the view that the Australian government should ask the MSAC to review the criteria for reimbursement of ongoing diagnostic tests such as MRIs. Given this testing is not discretionary but used to determine disease progression and treatment options, the committee believes it is appropriate for such ongoing diagnostics to be reimbursed.

Recommendation 22

5.179 The committee recommends that the Australian government asks the Medical Services Advisory Committee to review the criteria for reimbursement of ongoing diagnostic testing for low survival rate cancer patients.

5.180 The government should also address the barriers and time delays encountered by LSR cancer patients and their families when seeking financial support such as the DSP or the carer allowance or payment. As the committee has already highlighted, burdening LSR cancer patients and their families with unnecessarily complex

administrative processes and time delays—especially where a person has a terminal diagnosis and time is precious—is inappropriate. The committee therefore recommends that the Australian government further simplifies and streamlines the application processes for LSR cancer patients and their carers when seeking to access the DSP or carer allowance or payment.

Recommendation 23

5.181 The committee recommends that the Australian government further simplifies and streamlines the application process for low survival rate cancer patients and their carers when seeking to access the Disability Support Pension, or carer allowance or payment.

A national strategy for people with low survival rate cancers

5.182 The following sections of this report examine:

- the work of the Australian government to date developing and implementing a national approach to cancer;
- recent announcement for a plan to increase the rate of survival for people with brain cancer; and
- some key international developments.

5.183 The final section considers a proposal for a national strategy to increase survival rates for all LSR cancers in Australia.

The National Cancer Work Plan

5.184 In April 2010, the Council of Australian Governments (COAG) agreed that:

> Victoria and the Commonwealth would lead work under the auspices of Health Ministers, to report back to COAG in 2011, on the most effective cancer diagnosis, treatment and referral protocols, to be developed with expert clinical input[168]

5.185 Subsequently, the National Cancer Expert Reference Group (NCERG), jointly chaired by the Australian and Victorian governments, was formed, comprising 'senior representatives of all jurisdictions and peak stakeholder bodies ([COSA]; [CCA]; Cancer Australia; and consumer representation)'.[169]

5.186 In July 2012, the NCERG released a National Cancer Work Plan (the Plan), described as:

> …a suite of initiatives, focused on providing appropriate, efficient and well coordinated care for people affected by cancer and their families, from

168 National Cancer Expert Reference Group (NCERG), *COAG Improving Cancer Care Initiative National Cancer Work Plan*, July 2012, p. 1.

169 NCERG, *COAG Improving Cancer Care Initiative National Cancer Work Plan*, July 2012, p. 1.

diagnosis through treatment and support to the management of follow-up care and survivorship.[170]

5.187 The three key initiatives of the Plan are:

- *Initiative 1 – Pathways of cancer care* which will:

  a) establish best-practice pathways of cancer care with agreed referral protocols (including post-treatment and survivorship) between GPs, cancer specialists and other allied health professionals; and

  b) improve the practical support available to patients, their carers and families so that they can better navigate the complex cancer journey.[171]

- *Initiative 2 – Efficient and effective cancer services*, to 'be achieved by working with consumers, jurisdictions and peak health professional bodies to establish':

  a) the piloting of innovative use of the cancer workforce including service efficiencies, scope of practice, and new models of shared care for cancer treatment; and

  b) agreed capability frameworks for cancer services with defined linkages to primary care, regional cancer services and specialist tertiary teaching hospitals, and the promotion of safe, high quality cancer care by agreed role delineation for cancer services, specific tumours and sub-specialties to optimise outcomes.[172]

- *Initiative 3 – Evidence-based cancer treatment*, which will promote:

  a) better use of multidisciplinary initial assessment and treatment planning cancer teams across both the public and private sector. The new National Broadband Network and tele-health technology will be used to support multi-disciplinary care in regional areas where feasible; and

  b) the implementation of new research findings, evidence-based treatment and care, commencing with the national adoption of the NSW Cancer Institute's eviQ database as an easily accessible, consistent, on-line, point-of-care treatment resource for cancer health professionals.[173]

5.188 The Plan also contains the following agreed principles:

1. Focus on actions that require national coordination rather than those that can be achieved by one level of government alone; build upon existing

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170 NCERG, *COAG Improving Cancer Care Initiative National Cancer Work Plan*, July 2012, p. 1.

171 NCERG, *COAG Improving Cancer Care Initiative National Cancer Work Plan*, July 2012, p. 2.

172 NCERG, *COAG Improving Cancer Care Initiative National Cancer Work Plan*, July 2012, p. 2.

173 NCERG, *COAG Improving Cancer Care Initiative National Cancer Work Plan*, July 2012, p. 2.

> jurisdictional cancer plans and enhance the current investments made by all governments within reasonable timeframes.
>
> 2. Be underpinned by best-practice cancer research and optimal, evidence-based cancer treatment and supportive care.
>
> 3. Recognise the fiscal outlook facing all governments and the difficulty of funding significant new activity, and focus on high-impact and achievable actions.[174]

5.189 Cancer Australia, on behalf of the Australian government, implements the following components of the Plan:

- a dedicated cancer research budget
- support for cancer clinical trials
- the 'Supporting people with cancer' program
- the Improved lung cancer data and treatment guidelines measure.[175]

5.190 Cancer Australia spoke to a few of these components, but did not refer to the Plan itself. For example, in respect of the 'supporting people with cancer' component, Cancer Australia informed the committee that it works with local communities, funding them 'to potentially raise awareness or to provide supportive care to their communities'.[176]

5.191 Notably, the NCERG's 'future directions' for the 2016–2017 financial year did not specifically address LSR cancers:

> In 2016-17, NCERG will consolidate work undertaken to date in implementing the National Cancer Work Plan and continue to provide a crucial forum for coordination of cancer policy and control at a national level. The focus in 2016-17 will be on implementation of the [Optimal Cancer Care Pathways] and working with jurisdictions to encourage their uptake. This work will contribute to consistent cancer care across the country that maximises efficiencies and builds on the considerable recent investment in cancer infrastructure by all governments.[177]

5.192 The committee received no detailed information from the DoH or the NHMRC about how the Plan responds to LSR cancers.

5.193 While the Australasian Gastro-Intestinal Trials Group (AGITG) noted the work that Cancer Australia is doing to implement the Plan, it nevertheless recommended a national cancer research plan to specifically address LSR cancers:

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174 NCERG, *COAG Improving Cancer Care Initiative National Cancer Work Plan*, July 2012, p. 1.

175 Cancer Australia, *Grants and funding*, https://canceraustralia.gov.au/research-data/grants-and-funding (accessed 16 October 2017).

176 Adjunct Associate Professor Christine Giles, Executive Director, Cancer Australia, *Committee Hansard*, 29 August 2017, p. 23.

177 NCERG, *COAG National Cancer Work Plan Progress Report 2014-15 and 2015-16*, p. 11.

> We are aware of several of our States which have produced comprehensive Cancer Care Plans with stakeholder/consumer input. We are also aware that Cancer Australia has a National Cancer Care Plan and has allocated several initiatives to its [NCERG] one of these initiatives is "Evidence-based care for lung cancer - better lung cancer care - led by Cancer Australia." Whilst this initiative is comforting it is not what the AGITG [Consumer Advisory Panel] considers a comprehensive National Cancer Research Plan which should, inter alia, include specific research requirements for "low survival cancers".[178]

5.194 Indeed, as discussed further in a later section, a number of other submitters and witnesses called for a plan or strategy to specifically address the low rates of survival for LSR cancers.

A new strategy for combatting brain cancer

5.195 On 29 August 2017, the Hon. Greg Hunt MP announced the establishment of the Australian Brain Cancer Mission (the Mission), a $100 million fund to combat brain cancer, which:

> …aims to double survival rates of people living with brain cancer over the next 10 years, which hasn't changed significantly in the past 30 years.
>
> In the long-term our goal is to defeat brain cancer through world-wide collaboration.[179]

5.196 The Mission:

> …is underpinned by a research roadmap developed by Australian and international experts in brain cancer treatment and research, and those affected by brain cancer, their advocates and philanthropic interests.[180]

5.197 The Mission will be administered by Cancer Australia, which will be supported in this work by a Strategic Advisory Group.[181]

5.198 The minister noted that one of the key objectives of the Mission 'is to ensure every patient, adult and child in Australia has the opportunity to participate in clinical trials'.[182] To achieve this, the government will provide $50 million to the MRFF, which will be supplemented by $20 million from the CBCF and $10 million from the Minderoo Foundation's Eliminate Cancer Initiative.[183] The government will also dollar

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178 Australasian Gastro-Intestinal Trials Group, *Submission 85*, p. 4.

179 The Hon. Greg Hunt MP, 'Australian Brain Cancer Mission', *Media Release*, 29 October 2017, p. 1.

180 The Hon. Greg Hunt MP, 'Australian Brain Cancer Mission', *Media Release*, 29 October 2017, p. 1.

181 The Hon. Greg Hunt MP, 'Australian Brain Cancer Mission', *Media Release*, 29 October 2017, p. 2.

182 The Hon. Greg Hunt MP, ' Australian Brain Cancer Mission', *Media Release*, 29 October 2017, p. 1.

183 The Hon. Greg Hunt MP, 'Australian Brain Cancer Mission', *Media Release*, 29 October 2017, p. 1.

match 'every donation up to $50 million' to support the mission.[184] The government expects to 'announce the remaining $20 million in the coming months'.[185]

5.199 The '[p]rioritised first investments include the establishment of an Australian arm of the GBM AGILE, an international adaptive trial platform for adults with glioblastoma' and 'new funding for [ANZCHOG] clinical trial centres and support [for] the consolidation of the national ZERO Children's Cancer initiative'.[186]

International approaches to LSR cancers

5.200 During the course of the inquiry, the committee received evidence that specifically identified the US *Recalcitrant Cancer Research Act of 2012* (the Act) as an example of how governments can work to increase survival rates for LSR cancers.[187]

5.201 The Act '[a]mends the Public Health Service Act to require the Director of the National Cancer Institute (NCI) to develop a scientific framework for research on recalcitrant cancers (cancer with a 5-year relative survival rate below 50%)'.[188]

5.202 The framework for research includes:

> (1) a review of the status of research, such as a summary of findings, identification of promising scientific advances, a description of the availability of qualified scientific researchers, and the identification of resources available to facilitate research;
>
> (2) identification of research questions that have not been adequately addressed; and
>
> (3) recommendations for actions to advance research and for appropriate benchmarks to measure progress on achieving such actions. Requires the Director to develop the framework within 18 months and review and update it every 5 years.

5.203 The framework for research also requires the following actions of the Director of the NCI:

> …to identify within 6 months 2 or more recalcitrant cancers that have a 5-year relative survival rate of less than 20%, and are estimated to cause the death of at least 30,000 individuals in the [US] per year. Authorizes the Director to identify additional such cancers and to consider additional

184 The Hon. Greg Hunt MP, 'Australian Brain Cancer Mission', *Media Release*, 29 October 2017, p. 1.

185 The Hon. Greg Hunt MP, 'Australian Brain Cancer Mission', *Media Release*, 29 October 2017, p. 1.

186 The Hon. Greg Hunt MP, 'Australian Brain Cancer Mission', *Media Release*, 29 October 2017, p. 2.

187 See, for example, Pancare Foundation, *Submission 9*, p. 2; CanTeen Australia, *Submission 128*, p. 6; Ovarian Cancer Australia, *Submission 242*, p. 4.

188 Congress.gov, *H.R.733 - Recalcitrant Cancer Research Act of 2012*, https://www.congress.gov/bill/112th-congress/house-bill/733 (accessed 26 October 2017).

> metrics of progress (such as incidence and mortality rates) against such cancer.
>
> …to convene a working group for each identified cancer to provide expertise on, and assist in developing, a scientific framework under this Act.
>
> …to consider each relevant scientific framework developed under this Act when making recommendations for exception funding for grant applications.[189]

5.204 Although certain groups in the US, such as the Lung Cancer Alliance and the Pancreatic Cancer Action Network cautiously welcomed the Act as a result of their lobbying,[190] '[a]dvocates for other kinds of cancer research view [the Act] warily':

> A man named Jonathan Agin, who lost a small daughter to a kind of brain cancer with no treatment at all, has been a vocal critic both of the Act and of the NCI. When he met with representatives of NCI to argue for more funding of children's cancers, he was told that *funding allocation does not matter*, because discoveries in the lab often apply to many cancers.
>
> …
>
> It's also the case that the head of the NCI, Dr. Harold Varmus, is unhappy with the law because he believes it ties the hands of scientists to determine how money is spent. But others are unhappy with NCI and think there should be less emphasis on the search for cures and more emphasis on prevention. NCI's annual budget requests include billions for research and treatment, but usually less than $300,000 for prevention and control. It is argued we are likely to have better results putting money into preventing cancers to begin with rather than continuing to sink nearly all of our anti-cancer money into looking for cures.[191]

5.205 CanTeen Australia supported the implementation of similar legislation in Australia, stating that the Act is:

> …an example of how legislative change can support meaningful coordinated effort to improve outcomes for cancer with low survival rates. It guides not only the establishment of priority frameworks, but the accountability mechanisms required to ensure progress, public availability

189 Congress.gov, *H.R.733 - Recalcitrant Cancer Research Act of 2012*, https://www.congress.gov/bill/112th-congress/house-bill/733 (accessed 26 October 2017).

190 See, Lung Cancer Alliance, *Recalcitrant Cancer Research Act*, http://www.lungcanceralliance.org/lung-cancer-advocacy/impact-to-date/lung-cancer-mortality-reduction-act/ (accessed 26 October 2017); and Pancreatic Cancer Action Network, *Recalcitrant Cancer Research Act*, https://www.pancan.org/get-involved/advocacy/recalcitrant-cancer-research-act/ (accessed 26 October 2017).

191 Barbara O'Brien, 'The MCA Blog: The Effect of the Recalcitrant Cancer Act on Cancer Research', 5 December 2014, *Mesothelioma.com*, https://www.mesothelioma.com/blog/authors/barbara/the-effect-of-the-recalcitrant-cancer-act-on-cancer-research.htm (accessed 26 October 2017).

> requirements and how frameworks should be utilised to inform funding decisions.[192]

5.206 The Pancare Foundation also advocated for a national government commitment mirroring the approach taken in the US, and commended the approach to improving survival rates for pancreatic cancer currently under development in the UK.[193] Mr Barry Westhorpe, Chief Executive Officer of the Pancare Foundation, informed the committee that the UK All-Parliamentary Group on Pancreatic Cancer is looking at 'terms of reference based on a framework similar to the US model, not regulatory as such'.[194]

5.207 With regard to developments in brain cancer in the UK, TBTC informed the committee that, following a parliamentary inquiry into the funding for brain tumours:

> …the Department of Health was instructed to set up a task and finish working group to look at this issue. That working group has been taking evidence for probably about six months now, and the report is due out…there have been inputs to that across the board from drug discovery symptoms and various other things.[195]

5.208 TBTC suggested that the work of this committee 'will be a similar sort of piece' to what is currently happening in the UK in respect of brain cancer.[196] The UK report is yet to be published.

A national strategy for all LSR cancers?

5.209 As discussed earlier, the National Cancer Work Plan has no specific reference to LSR cancers. Further, LSR cancers were not identified as a specific priority of the NCERG for the 2016–2017 financial year. As the Low Survival Cancers Alliance has observed, Cancer Australia's Strategic Plan contains no focus on LSR cancers, supporting the Alliance's statement that 'there has been no ownership for responsibility for low survival cancers research at a Federal or state level'.[197]

5.210 Indeed, while COAG did consider a National Rare Diseases Plan in 2013, a recommendation for such a plan was ultimately not supported.[198]

5.211 A number of submitters and witnesses called for a plan or strategy to be established specifically to improve survival rates for LSR cancers. Some of these proposals are discussed in the following section.

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192 CanTeen Australia, answers to questions on notice, 19 May 2017, (received 9 June 2017), p. 5.

193 Mr Barry David Westhorpe, Chief Executive Officer, Pancare Foundation Inc., *Committee Hansard*, 7 June 2017, p. 50.

194 Mr Westhorpe, Pancare Foundation Inc., *Committee Hansard*, 7 June 2017, p. 53.

195 Dr Jenkinson, TBTC, *Committee Hansard*, 29 August 2017, p. 40.

196 Dr Jenkinson, TBTC, *Committee Hansard*, 29 August 2017, p. 40.

197 Low Survival Cancers Alliance, *Submission 90*, p. 4.

198 Research Australia, *Submission 122*, p. 20.

A new research plan

5.212 As the Pancare Foundation highlighted, 'there isn't a national strategic plan to increase survival, nor a definition on what constitutes cancers with low survival rates'.[199] It was submitted that, in terms of research, '[t]his translates into uncoordinated plans instead of a long term, national coordinated approach across the government, medical, health and research communities'.[200]

5.213 Other submitters and witnesses called for a national strategy to address funding of research into LSR cancers. For example, Mr Chiem advocated for a '[n]ational strategy to coordinate planning and funding of cancer research and reduce the associated administrative overheads', reasoning that:

> This will minimise duplication of efforts and reduce the highly bureaucratic and administrative overhead of research/grant application and reporting. Funding agencies should also partner to fund like-areas and capitalise on the economies of scale afforded by the joint funding. A particular study found that the time spent to prepare for NHMRC proposal translated into annual salary costs of $66 million. Furthermore, as success rates of NHMRC grant proposal outcomes are historically 20-25%, there are large opportunity costs in lost research output.[201]

5.214 Speaking particularly to brain cancer, Mr Barrie Littlefield of CBCF informed the committee that the mission of the CBCF is to 'increase brain cancer survival from the current 20 per cent to 50 per cent by 2023', and stated that:

> We need a firm, coordinated plan around this mission. Whilst more money for research is important, it is also important that the delivery and allocation of this money when it comes is coordinated, working to a clear, agreed plan based on our mission, hopefully, both here and internationally. Australia needs to work to its strengths, do what it does well and not repeat what is being done elsewhere. It is unlikely that Australia alone will cure brain cancer, but it can and should play its part.[202]

5.215 Cancer Voices Australia (CVA) opined that 'without a plan we have no idea where things are headed' and suggested that a national strategic plan for cancer research would provide greater transparency to consumers and specialists about the allocation of funding for research.[203] CVA detailed its proposal:

> Such a document, co-designed by key cancer survivors, researchers and health care providers, would provide greater transparency of the focus and priorities for research funding. In the absence of a national plan, the current model of funding is not equitable in allocating funding to cancers with low

199 Pancare Foundation, *Submission 9*, p. 1.

200 Pancare Foundation, *Submission 9*, p. 1.

201 Mr Chiem, *Submission 110*, p. 3 (citations omitted).

202 Mr Barrie Littlefield, Head of Engagement, CBCF, *Committee Hansard*, 6 June 2017, p. 22.

203 Ms Christine Christensen, Chair, Cancer Voices South Australia, and Executive Member, Cancer Voices Australia (CVA), *Committee Hansard*, 18 May 2017, p. 16.

> survival rates and has resulted in a limited evidence base for these cancers. A National Cancer Research Plan should embed funding into cancers with low survival rates and require the establishment of a register for each cancer. This should include funding for the multiple and cumulative reasons for low survivor rates, for example, late or incorrect diagnosis, lack of access to appropriate therapies and clinical expertise, the very limited number of clinical studies due to the small number of patients and the apparent lack of interest in developing new therapies due to market limitations.
>
> A national plan should include targets for research into cancers which currently have low survival rates, while at the same time providing a national focus for research into all cancers. A national plan should also support collaborative, baseline work, so necessary in identifying and prioritising gaps in research with consumers, researchers and health care providers to set research actions plans for cancers with low survival rates. Annual reporting to Parliament on progress towards targets in the plan should be mandatory. In addition it is recommended that the Australian Institute of Health and Welfare establish routine reporting of the category 'cancers with low survival rates' to collectively report on the incidence and overall proportion of mortality contributed by this group, and to track positive or adverse changes within this group. It is also suggested that as part of this reporting rare cancers and higher incidence, but low survival cancers, are separately reported.
>
> A National Cancer Research Plan and associated registry could provide information to the public about sites where research into cancers with low survival rates is occurring so that cancer survivors, their carers and the public can access information about treatment options, and cancer researchers can see opportunities for collaboration and/or innovation. Cancer Voices believes a new funding model [should] address identified unmet needs and move away from clinical trials that propose marginal improvement in care, particularly as more subsets of cancers are identified.[204]

5.216 CanTeen Australia suggested that the NHMRC could be charged with developing:

> …a scientific framework or multiple frameworks to guide the conduct and funding of research for the cancers with both low survival rates and low representation in funding distributions to date. As an organisation, the NHMRC may be best placed to develop such a framework given its prominent role in shaping the Australian medical research landscape and working collaboratively to establish nationally applicable frameworks such as the 2007 National Statement on Ethical Conduct in Human Research.[205]

5.217 CanTeen Australia considered that a collaborative, representative body could achieve a national strategy for improving outcomes for LSR cancers, and outlined that

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204 CVA, *Submission 61*, pp 2–3.

205 CanTeen Australia, answers to questions on notice, 19 May 2017, (received 9 June 2017), p. 5.

such a strategy would assist the NHMRC and MRFF in setting their funding priorities.[206]

5.218 Similar to CVA, CanTeen Australia advocated for a new framework that 'could include clear accountability mechanisms for monitoring progress on the strategy and similar requirements for public availability of these strategies'.[207]

Committee view

5.219 The committee welcomes the government's recent funding announcements, and is particularly encouraged by the investment of $100 million for a 10 year plan to increase the survival rates for brain cancer. This illustrates the government's understanding that funding for research is inextricably linked to increasing survival rates for cancers.

5.220 The committee is concerned, however, by the continued absence of explicit recognition of LSR cancers, in terms of funding and in government plans to address cancer in Australia.

5.221 As a result, the committee considers it necessary for a comprehensive Australia-wide strategy to be developed and implemented to address LSR cancers, with the explicit goal of increasing the 5-year survival rates for LSR cancers to above 50 per cent by 2027. The development of such a strategy will require the participation and commitment of the federal, state and territory governments, and could be developed via the NCERG and COAG.

5.222 The development of an Australian strategy to improve survival rates for LSR cancers should take into account the recommendations in this report; must consult with medical researchers, clinicians, patients and patient groups; and consider the roles of research, early diagnosis and access to medicines. International approaches, such as the *Recalcitrant Cancer Research Act of 2012 (US)*, should also be considered and an assessment made as to whether similar legislation is appropriate in the Australian context.

Recommendation 24

5.223 The committee recommends that the federal, state and territory governments develop and implement a comprehensive Australia-wide strategy to increase 5-year survival rates for low survival rate cancers to above 50 per cent by 2027:

- **taking into account the recommendations in this report;**
- **consulting with researchers, clinicians, patients and patient groups;**
- **considering the roles of research, early diagnosis and access to medicines; and**

206 CanTeen Australia, answers to questions on notice, 19 May 2017, (received 9 June 2017), p. 5.

207 CanTeen Australia, answers to questions on notice, 19 May 2017, (received 9 June 2017), p. 5.

- **assessing the applicability of international approaches, such as the *Recalcitrant Cancer Research Act of 2012 (US)*, to the Australian context.**

5.224 The committee further recommends that annual progress reports on the development and implementation of an Australian strategy to improve survival rates for LSR cancers are provided to COAG's Health Council and made publicly available.

Recommendation 25

5.225 The committee recommends that annual progress reports on the development and implementation of an Australian strategy to improve survival rates for low survival rate cancers are provided to the Council of Australian Governments Health Council and made publicly available.

Senator Catryna Bilyk
Chair

Senator David Bushby
Deputy Chair

Senator Brian Burston
Pauline Hanson's One Nation, NSW

Senator Stirling Griff
Nick Xenophon Team, SA

Senator Jane Hume
Liberal Party of Australia, VIC

Senator Chris Ketter
Australian Labor Party, QLD

Senator Malarndirri McCarthy
Australian Labor Party, NT

Senator Dean Smith
Liberal Party of Australia, WA

Appendix 1

Submissions and additional information

Submissions

1 Ms Wendy Jackson

2 Mr Tom Murkin
2.1 Supplementary to submission 2

3 Ms Jacqueline Ohlin

4 Ms Melissa Iocco-Fischer

5 Mrs Judith Polkinghorne

6 Ms Christine Jones

7 Mrs Sandra Woods

8 Ms Linda Ferguson

9 Pancare Foundation

10 Ms Dianne Pooley

11 Mrs Emma Bhatti

12 Mr Troy Bevan

13 Mrs Michelle Patterson

14 Mrs Natalie Bunworth

15 Mr Brendan Spain

16 Mrs Amanda Marriott

17 Ms Bonnie Palmer

18 Mrs Suzanne Turpie

19 Mrs Margaret Shonk

20 Mrs Judith Hearn
20.1 Supplementary to submission 20

21 Ms Lisa Wolker

22 Mrs Victoria Bushby

23 Ms Fiona Smith

24 Mr Stefan Testi

25 Ms Raquel Oliveira

26 Ms Karen Lloyd

27 Dr Sigrid Denehey

28 Mr Derek Maule

29 Mrs Rita Potenza

30 Asbestos Council of Victoria/GARDS
31 Mr Alex Cullen
32 Ms Jacqueline Walling
33 Mrs LeShae Harrison
34 Garvan Institute of Medical Research/Garvan Research Foundation
35 Mrs Madeline Bishop
36 Ms Helen Atkinson
37 Mrs Marj Salter
38 Mr David Stratton
39 Mr Graham Wells
40 Mrs Isabella Borghese
41 Mrs Marcella Zemanek
42 Mrs Joanna Wilson
43 Mr Tim Eliot
44 Mrs Teresa Briggs
45 Mr Dustin Perry
46 Mrs Therese Townsend
47 Mrs Natalie Wainman
48 UNSW Sydney & SPHERE
49 Centre for Community-Driven Research
50 Rare Cancers Australia
51 Professor Guy Eslick
52 Mrs T. Taylor
53 Mrs Raechel Burgett
54 Mrs Deborah Dunkley
55 Mrs Julie Brown
56 Mr Brendan Donohoe
57 Mr Thomas Smith
58 Ms Laura Kennedy
59 Ms Nadine Walsh
60 Brain Cancer Discovery Collaborative
61 Cancer Voices Australia
62 Mr Philippe Pierson
63 Mr Jason Spriggs
64 Mr Steven Coote
65 Mrs Kate Peacock

66 Mrs Maureen Fogarty
67 Mr Paul Jones
68 Ms Helen Cassidy
69 Mrs Kathryn Winsor-Harris
70 Mrs Sue Hohnke
71 Ms Georgina O'Brien
72 Mrs Yvonne Anthoney
73 The Cure Starts Now (Australia)
74 Prof. Richard Scolyer, Melanoma Institute Australia
75 Ms Kate Wenban
76 Ms Ruth Churchill
77 Mrs Lyndal Lean
78 Ms Elizabeth Perry
79 Mr Brett Withington
80 QIMR Berghofer Medical Research Institute
81 Ms Julia Barker
82 Mrs Susan Kay
83 Australian Institute of Health and Welfare
83.1 Supplementary to submission 83
84 Ethan Davies Fellowship
85 Australasian Gastro-Intestinal Trials Group
86 Ms Julie Butel
87 National Health and Medical Research Council (NHMRC)
88 Children's Cancer Research Unit, The Children's Hospital at Westmead
89 Lung Foundation Australia
90 Low Survival Cancers Alliance
91 Mrs Jane Gordon
92 Mr Andrew Jakeman
93 Miss Lolita Rahi
94 Mr Paul Bird
95 Ms Tanya Cardamone
96 Mr Justin Benson
97 Mrs Sheree Gover
98 Mr Denis Strangman AM
98.1 Supplementary to submission 98
99 Mr Jonathan Karl Fretwell
100 Mrs Rachel Furniss

101 The Unicorn Foundation
102 Pancreatic Cancer Alliance
103 Thoracic Society of Australia and New Zealand (TSANZ)
103.1 Supplementary to submission 103
104 Associate Professor Prue Cormie
105 Mr Jon May
106 Mrs Jessica Bennett
107 Mr Brian Holloway
108 Ms Michelle Bradley
109 Ms Barbara Purcell
110 Mr Khang Chiem
111 Mrs Kellie Carroll
112 Medical Oncology Group of Australia
113 Mark Hughes Foundation
114 Victorian Comprehensive Cancer Centre
115 MSD Merck Sharp & Dohme (Australia) Pty Ltd
116 Mrs Carly Gray
117 Mr Simon Gray
118 Mr Martin Lister
119 Brain Cancer Biobanking Australia
120 LOVE FOR LACHIE
121 Australasian Leukaemia & Lymphoma Group
122 Research Australia
123 NSWOG Neuro-oncology
124 Roche Products Pty Limited
125 ANZCHOG National Patient and Carer Advisory Group
126 The Walter and Eliza Hall Institute of Medical Research
127 Brain Tumour Alliance Australia
128 CanTeen Australia
129 Cancer Australia
130 Sydney Neuro-Oncology Group
131 The Isabella and Marcus Paediatric Brainstem Tumour Fund
132 The University of Newcastle and Hunter Medical Research Institute
133 Queensland Brain Institute (UQ)
134 Rare Voices Australia
135 Braver Stronger Smarter Inc
136 The Kids' Cancer Project

137 Cancer Council Australia & the Clinical Oncology Society of Australia
138 Cooperative Trials Group for Neuro-Oncololgy (COGNO)
139 Cure Brain Cancer Foundation
140 checkyourtackle
141 Medicines Australia
142 Mr Andrew Hamilton
143 Ms Belinda Peden
144 Mr Graham Smith
145 Mrs Christine Goodman
146 Mr Steven Neller
147 Ms Anne Murphy
148 Ms Jessica Annetts
149 Ms Susanne Casimaty
150 Mr Benjamin Leske
151 The Adnum Family
152 Mrs Bridget Marker
153 Miss Kaela Gray
154 Mrs Kathy Farrell
155 Mrs Leah Lane
156 Mr Bruce Stewart
157 Mr Anthony Gray
158 Mrs Jennifer Swain
159 Mrs Sarah McGoram
160 Mrs Dominique Holden
161 Professor Mary-Louise McLaws
162 Miss Melanie Johnston
163 Mr Myles Brewster
164 Ms Lyn Barker
165 Mr Thomas Greenaway
166 Mrs Mary Cranston
167 Australia and New Zealand Melanoma Trials Group
168 Mrs Fleur Kay
169 Mrs K. Webster
170 Ms Robin Berthelsen
171 Ms Brianna Armstead
172 Mrs Katherine Landers

173 Ms Kathie Griffiths
174 Mr Nick Lowman
175 The Dullard Family
176 Mr Gino Iori
177 Mr Leo Harlond
178 Mrs Soo Fong Hamilton
179 Mrs Amanda Stork
180 Mrs Lyndall Bates
181 Mrs Pauline Stanistreet
182 Mr Norman Stanistreet
183 Mr Hugo Zweep
184 Mr Robert Perkins
185 Mrs Karyn Harris
186 Ms Anna Kelly
187 Cancer Drugs Alliance
188 Ms Anne Cashman
189 Ms Diane Dunn
190 Ms Morgana Ryan
191 Ms Kylie Newton
192 Ms Evangeline Lim
193 Mr Jake Briggs
194 Ms Helen Jones
195 Ms Leanne Lindsay
196 Ms N. Sheehan
197 Mr Geoff Webster
198 Ms Sophie Briggs
199 Mr Manuel Graeber
200 Mr Ward Jongebloed
201 Mr Michael Buckland
202 Ms Andrea Ward
203 Ms Krystyna Zerger-Daddow
204 CSIRO
205 School of Public Health and Preventive Medicine, Monash University
206 Mrs Deirdre Ross
207 Mr Tom Akhurst
208 Mrs Rebecca Bidstrup

209 Ms Belinda Suttor
210 Ms Cynthia Sargent
211 Mrs Faith Mantzounis
212 Mr Tom Wood
213 Miss Maddy Clohessy
214 Mrs Natasha Osborne
215 Mrs Sara Wilson
216 Dr Fiona Reid
217 Mrs Lisa Harries
218 Mrs Catherine Mitchell
219 Mrs Jackie Barreau
220 Mrs Debra Ponder
221 Mr Ian Becker
222 Mr Donald Kennedy
223 Mrs Tami Collins
224 Mrs Rose McKenzie
225 Mr Sydney Rudd-Schmidt
226 Ms Kylie Rupil
227 Mr Daniel Robinson
228 Mr Christopher Hicks
229 Dr Rachel Harris
230 Mrs Lisa Judd
231 Mrs Michelle Armstrong
232 Ms Susan Pitt
233 Mr Leigh Armstrong
234 Ms Monika Schmidt
235 Mr Cameron Rowick
236 Miss Elyse Chant
237 Australian and New Zealand Children's Haematology and Oncology Group (ANZCHOG)
238 Mrs Helen Pegler
239 Mr Sam Heyes
240 Mr Phil Reynolds
241 Ms Marilyn Nelson
242 Ovarian Cancer Australia
243 Robert Connor Dawes Foundation
244 Bourne and Associates

245 Mr William Williams
246 Mr Colin Bienke
247 Ms Suzanne Cahill
248 Ms Anna Rothwell
249 Name Withheld
250 Mr Frane Vidovic
251 Mrs Stephanie Vaughan
252 Professor Terrance Johns
253 Ms Caroline Greenaway
254 Mr Matthew Hirst
255 Ms Denise Brown
256 Mrs Anna-Marie Griffith
257 Ms Nyree Waterson
258 Mrs Emilie Prendergast
259 Mrs Amanda Fintan
261 Ms Poppy Mosses
262 Mr Richard Hoopmann
263 Mrs Carol Rodgers
264 Ms Catherine Cantlon
265 Ms Frances Burrows
266 Ms Clare Stuparich
267 Mr Tony La Ferrara
268 Ms Sarah Mamalai
269 Professor David Walker
270 Confidential
271 Centre for Cancer Biology
272 Ms Katrina Hill
273 Mrs Alice Findlay
274 Children's Hospital Foundation
275 Mr Michael Mestrovic
276 Ms Sherrin Bell
277 Ms Rhonda Stubbins
278 Mr Peter Ramstadius
279 Ms Annabelle Wilson
280 Children's Hospital Foundation/Australian Centre for Health Services Innovation
281 Ms Jo Justo

282 Dr Andrew Haydon (an identical submission was also received from Dr Alan Pham)
283 Mr Tony Monaghan
284 Ms Jo Kuropatoff
285 Ms Louise Casamento
286 Confidential
287 Mr John Hackett
288 Mr Martin Shafron
289 Bristol-Meyer Squibb
290 Name Withheld
291 Name Withheld
292 Name Withheld
293 Name Withheld
294 Name Withheld
295 Name Withheld
296 Name Withheld
297 Name Withheld
298 Name Withheld
299 Name Withheld
300 Name Withheld
301 Beyond Five
302 Name Withheld
303 Name Withheld
304 Name Withheld
305 Name Withheld
306 Name Withheld
307 Name Withheld
308 Name Withheld
309 Name Withheld
310 Name Withheld
311 Name Withheld
312 Name Withheld
313 Confidential
314 Confidential
315 Confidential
316 Confidential
317 Confidential

318 Confidential

319 Confidential

320 Confidential

321 Ms Paula McKelvie

322 Mrs Jessica Nightingall

323 Mr Roger Renshaw

324 Mr Scott Thomson

325 Ms Rachel Cutler

326 Mrs Trudy Fittler

Tabled documents

1 Documents tabled by the Australian Gastro-Intestinal Trials Group at public hearing on 18 May 2017 - Research Infrastructure Funding

2 Document tabled by Associate Professor Michael Buckland at public hearing on 18 May 2017 - The Utility of Expert Diagnosis in Surgical Neuropathology

3 Documents tabled by Ms Marilyn Nelson at public hearing on 6 June 2017 - Documents

4 Document tabled by Ms Catherine Peacock at public hearing on 7 June 2017 - Document

5 Document tabled by Professor David Thomas at public hearing on 8 June 2017 - Document

6 Documents tabled by the Brain Tumour Alliance Australia at public hearing on 8 June 2017 - List of documents tabled

7 Document tabled by Ovarian Cancer Australia at a public hearing on 4 August 2017 - National action plan for ovarian cancer research

8 Document tabled by Department of Health at a public hearing on 29 August 2017 - Medical Research Future Fund, Lifting Clinical Trials and Registries Capacity (LCTRC) Grant Guidelines

9 Document tabled Department of Health at a public hearing on 29 August 2017 - Medical Research Future Fund: Australian Medical Research and Innovation Strategy 2016-2021 and Australian Medical Research and Innovation Priorities 2016-2018

10 Document tabled by the Department of Health at a public hearing on 29 August 2017 - Medical Research Future Fund information sheets

Answers to questions on notice

1 Australian Charities and Not-for-profits Commission (ACNC) - answer to a written question on notice (received 27 February 2017)

2 CanTeen Australia - answers to questions taken on notice from public hearing 19 May 2017, Sydney (received 9 June 2017)

3 Australian Institute of Health and Welfare (AIHW) - answers to questions taken on notice from public hearing 8 June 2017, Canberra and written QoN dated 14 June 2017 (received 3 July 2017)

4 National Health and Medical Research Council (NHMRC) - answers to questions taken on notice from public hearing 29 August 2017, Canberra (received 19 September 2017)

5 Department of Health - answer to question taken on notice from public hearing 29 August 2017, Canberra (received 22 September 2017)

6 Cancer Australia - answer to question taken on notice from public hearing 29 August 2017, Canberra (received 22 September 2017)

7 Cancer Council Victoria - answers to questions taken on notice from public hearing 18 May 2017, Sydney (received 18 October 2017

8 Medicines Australia - answers to questions taken on notice from public hearing 8 June 2017, Canberra (received 20 October 2017)

Additional information

1 Summary of the Australian Genomic Cancer Medicine Program and costing breakdown

2 Garvan Institute - Proposal: national genomic cancer medicine program for rare and less common cancers (received 6 September 2017)

3 Finding Myself In Your Hands -The Reality of Brain Tumour Treatment and Care

4 Losing Myself - The Reality of Life with a Brain Tumour

Correspondence

1 Letter of correction to evidence given by Associate Professor Clare Scott at public hearing in Melbourne, 4 August 2017

2 Letter of correction to evidence given on behalf of Cancer Australia at the public hearing in Canberra, 29 August 2017

Appendix 2

Public hearings and witnesses

Monday, 18 May 2017—Sydney

ARMSTRONG, Mr James, Member, Consumer Advisory Panel, GI-Cancer Institute, Australasian Gastro-Intestinal Trials Group

BARREAU, Ms Jackie, Member, Cancer Voices South Australia

BUCKLAND, Associate Professor Michael, Private capacity

BUTOW, Professor Phyllis, President, Clinical Oncology Society of Australia

CANFELL, Professor Karen, Director of Research, Cancer Council NSW, Cancer Council Australia

CHRISTENSEN, Ms Christine, Chair, Cancer Voices South Australia, and Executive Member, Cancer Voices Australia

CONLEY, Mr Russell, Chief Executive Officer, Australasian Gastro-Intestinal Trials Group

CULLEN, Mr Alexander, Ambassador, Cure Brain Cancer Foundation

ESLICK, Professor Guy, Professor of Cancer Epidemiology and Medical Statistics, University of Sydney

FERGUSON, Ms Linda, Private capacity

GRAEBER, Professor Manuel, Barnet-Cropper Chair of Brain Tumour Research, Brain and Mind Centre, University of Sydney

HARPER, Mr Todd, Chief Executive Officer, Cancer Council Victoria, Cancer Council Australia

JOHNS, Professor Terrance, Director, Brain Cancer Discovery Collaborative

KENT, Mr Dan, Past Chair, Consumer Advisory Panel, Australasian Gastro-Intestinal Trials Group

KOH, Dr Eng-Siew, Deputy Chair, Management Committee, and Deputy Chair, Scientific Advisory Committee, Cooperative Trials Group for Neuro-Oncology, NHMRC Clinical Trials Centre, University of Sydney

MARKER, Ms Julie, Executive Teams, Cancer Voices Australia and Cancer Voices South Australia

MARKER, Ms Julie, Member, Consumer Advisory Panel, Australasian Gastro-Intestinal Trials Group

MUMFORD, Ms Jan, Member, Consumer Advisory Panel, GI-Cancer Institute, Australasian Gastro-Intestinal Trials Group

O'NEILL, Associate Professor Geraldine, Chief Investigator, Brain Cancer Discovery Collaborative

ROSENTHAL, Professor Mark, Chair, Cooperative Trials Group for Neuro-Oncology, NHMRC Clinical Trials Centre, University of Sydney

SHONK, Mr Evan, Private capacity

SHONK, Mrs Margaret, Private capacity

SIMES, Professor R John, Executive Member, Cooperative Trials Group for Neuro-Oncology; and Director, NHMRC Clinical Trials Centre, University of Sydney

TURPIE, Mrs Suzanne, Private capacity

VINES, Mr Richard, Chief Executive Officer, Rare Cancers Australia

WOODS, Mrs Sandra Joy, Private capacity

Friday, 19 May 2017—Sydney

BYRNE, Professor Jennifer Anne, Head, Children's Cancer Research Unit, the Children's Hospital at Westmead

KELSO, Professor Anne, Chief Executive Officer, National Health and Medical Research Council

KERR, Mrs Jane, General Manager, Thoracic Cancer and Rare Lung Disease, Lung Foundation Australia

LEIGH, Ms Lillian, Consumer/Lung Cancer Patient Advocate, Lung Foundation Australia

ORCHARD, Mr Peter, Chief Executive Officer, CanTeen Australia

PARKINSON, Dr Jonathon, Chair, New South Wales Oncology Group, Neuro-oncology

PAVLAKIS, Associate Professor Nick, President, Australasian Lung Cancer Trials Group, Lung Foundation Australia

PETERS, Professor Matthew, Professor Respiratory Medicine, Thoracic Society of Australia and New Zealand

STEIN, Dr Brian, Medical Oncology Group of Australia

Tuesday, 6 June 2017—Brisbane

ARMSTRONG, Ms Zoe, Clinical Research Director, Merck Sharpe & Dohme Australia

BUNT, Dr Jens, Research Fellow and Team Leader, NFI Research Lines, Brain Development and Disorders Laboratory, Queensland Brain Institute, The University of Queensland

BURGETT, Mrs Raechel, Private capacity

DAY, Dr Bryan William, Team Head, Translational Brain Cancer Research Laboratory, QIMR Berghofer Medical Research Institute

LIM, Mrs Evangeline, Private capacity

LITTLEFIELD, Mr Barrie, Head of Engagement, Cure Brain Cancer Foundation

NELSON, Ms Marilyn, Private capacity

RAYMOND, Ms Emma, Theme Leader, Cancer, Wesley Medical Research

RICHARDS, Professor Linda, Deputy Director, Research, Queensland Brain Institute, The University of Queensland

SELLARS, Mr Christian, Director, Market Access and Public Affairs, Merck Sharpe & Dohme Australia

SPITERI, Mr Carmel, Team Leader, Market Access, Merck Sharpe & Dohme Australia

STEWART, Ms Michelle, Head of Research Strategy, Cure Brain Cancer Foundation

TAYLOR, Mrs Tracy, Private capacity

WADDELL, Dr Nicola, Group Leader, Medical Genomics Group, QIMR Berghofer Medical Research Institute

WALKER, Professor David, Private capacity

WHITEMAN, Professor David, Deputy Director, QIMR Berghofer Medical Research Institute

Wednesday, 7 June 2017—Melbourne

AKHURST, Mr Tom, Private capacity

DE ROSE, Dr Robert, Co-founder, The Isabella and Marcus Paediatric Brainstem Tumour Fund

FRASER, Dr Chris, Chair, Australian and New Zealand Children's Haematology/Oncology Group

GOTTARDO, Clinical Associate Professor Nicholas, Deputy Chair, Australian and New Zealand Children's Haematology/Oncology Group; and Chair, Central Nervous System Tumour Subcommittee, Australian and New Zealand Children's Haematology/Oncology Group

GOULBURN, Mr Daniel, Member, Pancreatic Cancer Alliance

GRAY, Mr Simon, Private capacity

GRAY, Mrs Carly, Private capacity

HERTZBERG, Professor Mark, Member and Director, Australasian Leukaemia and Lymphoma Group

JOHNSON, Dr Elizabeth, Program Manager, Victorian Comprehensive Cancer Centre

KEMPEN, Mr Peter, Chairman of the Board, Australasian Leukaemia and Lymphoma Group

LEYDEN, Ms Simone, Chief Executive Officer and Co-founder, Unicorn Foundation

MILLIS, Mrs Nicole, Executive Officer, Rare Voices Australia

MULLINS, Mr Greg, Head of Policy, Research Australia

PAJIC, Dr Marina, Group Leader, Garvan Institute of Medical Research

PEACOCK, Mrs Catherine, Private capacity

PERRY, Mr Dustin, Private capacity

ROBERTS, Professor Andrew, Member and Director, Australasian Leukaemia and Lymphoma Group

ROSENTHAL, Professor Mark, Clinical Trials Lead, Victorian Comprehensive Cancer Centre

SMITH, Ms Delaine, Chief Executive Officer, Australasian Leukaemia and Lymphoma Group

STRONG, Ms Robyn, Executive Officer, Australian and New Zealand Children's Haematology/Oncology Group

WESTHORPE, Mr Barry David, Chief Executive Officer, Pancare Foundation Inc.

WRIGHT, Associate Professor Gavin, Research and Education Lead, Lung Cancer, Victorian Comprehensive Cancer Centre

Thursday, 8 June 2017—Canberra

AUNEDI, Mrs Helen, Country Head, Country Clinical Operations, Roche Australia Pty Ltd

BERMAN, Mrs Tricia, Secretary, Brain Tumour Alliance Australia

DE SOMER, Ms Elizabeth, Director of Policy and Research, Medicines Australia

FRAZER, Professor Ian, Chair, Australian Medical Research Advisory Board

GRADY, Dr Melissa, Regional Director, Site Management and Monitoring, Asia Middle East and Africa Clinical Operations, AstraZeneca

HAMPTON, Ms Blanche, Head, Strategy and Partnership, Garvan Institute of Medical Research

HARVEY, Mr Justin, Unit Head, Cancer and Screening, Health Group, Australian Institute of Health and Welfare

HINDSON, Mrs Catherine, Chair, Brain Tumour Alliance Australia

MOON, Dr Lynelle, Group Head, Health Group, Australian Institute of Health and Welfare

PARSONS, Mrs Alice, Committee Member, Brain Tumour Alliance Australia

PITT, Ms Susan, Private capacity

SHEEHAN, Ms Nicola, Committee Member, Brain Tumour Alliance Australia

STEEL, Mr Philip, Vice-Chair, Brain Tumour Alliance Australia

STRANGMAN, Mr Denis, AM, Private capacity

THOMAS, Professor David, Director, The Kinghorn Cancer Centre; Head, Cancer Research Division, Garvan Institute of Medical Research

WILLIAMS, Mr William (Billy), Private capacity

Friday, 4 August 2017—Melbourne

BLAKEMORE, Mrs Carolyn, Consumer, Australian and New Zealand Children's Haematology/Oncology Group National Patient and Carer Advisory Group

BRIGGS, Miss Sophie, Private capacity

BRIGGS, Mrs Teresa, Private capacity

DENEHEY, Dr Sigrid, Private capacity

EMBERSON, Ms Jill, Patient and Consumer Advocate, Ovarian Cancer Australia

FOX, Professor Stephen, Director of Pathology, Peter MacCallum Cancer Centre

HARPER, Mr Todd Andrew, Chief Executive Officer, Cancer Council Victoria

HIBBERT, Mr Laurence, Member, Australian and New Zealand Children's Haematology/Oncology Group National Patient and Carer Advisory Group

HILL, Ms Jane, Chief Executive Officer, Ovarian Cancer Australia

IOCCO-FISCHER, Ms Melissa, Private capacity

KAY, Ms Anne, Chair, Australian and New Zealand Children's Haematology/Oncology Group National Patient and Carer Advisory Group

KENNEDY, Ms Laura, Private capacity

LAMBERT, Dr Ken, Private capacity

ROBERTS, Professor Andrew, Head of Clinical Translation, Walter and Eliza Hall Institute of Medical Research

SCOTT, Professor Clare Scott, Head, Rare Cancer Research, Walter and Eliza Hall Institute of Medical Research

SEYMOUR, Professor John, Director, Department of Haematology, and Associate Director, Clinical Research, Peter MacCallum Cancer Centre

SLOCOMBE, Dr Judith, Chair, Consumer Advisory Panel, Walter and Eliza Hall Institute of Medical Research

WITHINGTON, Mr Brett, Private capacity

Tuesday, 29 August 2017—Canberra

BUTT, Dr Alison, Senior Scientific Officer, Cancer Australia

CREELMAN, Ms Alice, Assistant Secretary, Department of Health

DE ABREU LOURENCO, Dr Richard, Member, Australian Health Economics Society

GILES, Adjunct Associate Professor Christine, Executive Director, Cancer Australia

GLOVER, Dr Julie, Acting Executive Director, Research Programs, National Health and Medical Research Council

HARTLAND, Mr Nicholas, First Assistant Secretary, Research, Data and Evaluation, Department of Health

JACKSON, Dr Paul, Acting General Manager, Knowledge Management, Cancer Australia

JENKINSON, Dr David, Chief Scientific Officer, The Brain Tumour Charity

KELSO, Professor Anne, Chief Executive Officer, National Health and Medical Research Council

KNEIPP, Ms Erica, Assistant Secretary, Department of Health

REYNOLDS, Ms Leanne, Head of Research, Pancreatic Cancer UK

SHAKESPEARE, Ms Penny, First Assistant Secretary, Department of Health

VINEY, Professor Rosalie, Member, Australian Health Economics Society

WILSON, Professor Andrew, Chair, Pharmaceutical Benefits Advisory Committee