Chapter 1

Introduction

Access to cancer drugs in Australia

1.1 Australia is often described as the cancer capital of the world with the highest age-standardised incidence of cancer. Half of all Australians will develop cancer in their lifetime and one in five will die from it.[1] Australia also has cancer survival outcomes that are equivalent to the best in the world. Australia's one year survival rate for all cancers combined is 81 per cent and overall five year relative cancer survival rates are more than 66 per cent.[2] Together with investment in cancer detection and screening, investments in medical research have led to dramatic advances in the way cancer is treated and will be treated in the future.[3]

1.2 At the same time, there is widespread concern that Australian cancer patients continue to face significant delays and expense in accessing new cancer drugs, or existing drugs that are not available under the Pharmaceutical Benefits Scheme (PBS) for their form of cancer.[4]

The inquiry

1.3 On 3 December 2014, on the motion of Senator Nick Xenophon, the Senate referred the following matter to the Senate Community Affairs References Committee (committee) for inquiry and report by 26 March 2015:

> The availability of new, innovative and specialist cancer drugs in Australia, with particular reference to:
>
> (a) the timing and affordability of access for patients;
>
> (b) the operation of the Pharmaceutical Benefits Advisory Committee and the Pharmaceutical Benefits Scheme in relation to such drugs, including the impact of delays in the approvals process for Australian patients;
>
> (c) the impact on the quality of care available to cancer patients; and
>
> (d) any related matters.[5]

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1 Medicines Australia (MA), *Committee Hansard*, 20 April, p. 1.

2 Department of Health (DOH), *Submission 197*, p. 1.

3 Cancer Drugs Alliance (CDA), Submission 53, p. 1.

4 See: Herald Sun, *Melbourne woman pays $5k for drug PBS doesn't cover for her type of cancer*, 14 December 2014, http://www.heraldsun.com.au/news/victoria/melbourne-woman-pays-5k-for-drug-pbs-doesnt-cover-for-her-type-of-cancer/story-fni0fit3-1227155867412 (accessed 8 June 2015); News.com.au, Lifestyle, *Cancer sufferer Chris Brugger's family spend $16,000 every three weeks just to keep him alive*, 20 April 2015, http://www.news.com.au/lifestyle/health/cancer-sufferer-chris-bruggers-family-spend-16000-every-three-weeks-just-to-keep-him-alive/story-fneuzlbd-1227312367195 (accessed 8 June 2015)

5 *Journals of the Senate*, No. 73-3 December 2014, p. 1966.

1.4 On 9 February 2015, the Senate extended the reporting date to 22 May 2015.[6] The reporting date was subsequently extended to 17 September 2015.[7]

Conduct of the inquiry

1.5 The committee advertised the inquiry in *The Australian* on 15 January 2015. Details of the inquiry were placed on the committee's website and the committee wrote to 54 organisations, inviting submissions by 27 February 2015. Submissions continued to be accepted after that date. The committee received 205 submissions. A list of the individuals and organisations who made submissions to the inquiry is provided at Appendix 1.

1.6 A public hearing was held in Canberra on 20 April 2015. A transcript of the hearing is available on the committee's website,[8] and a list of the witnesses who gave evidence at the hearing is provided at Appendix 2. The committee thanks those individuals and organisations who contributed to the inquiry.

The structure of the report

1.7 Chapter 1 sets out the context of the inquiry. It provides an overview of the incidence of cancer in Australia and describes the regulatory pathway for the approval of medicines for marketing in Australia and reimbursement of the cost of some of those medicines through the PBS.

1.8 Chapter 2 examines the factors that affect the timing and affordability of access to new cancer medicines. It considers the operation of the TGA, the PBAC and the PBS.

1.9 Chapter 3 considers the PBAC's approach to the assessment of the cost and effectiveness of new cancer medicines as a prerequisite for listing on the PBS. It also considers the role that consumers and clinicians can play in this process.

1.10 Chapter 4 considers the impact of delays in the approvals process for Australian cancer patients. It examines the available pathways for access to cancer drugs not available through the PBS together with the need for timely and accurate information about new cancer medicines.

1.11 Chapter 5 examines some alternate models for facilitating access to new and innovative cancer drugs together with the need for improved data collection to support such models.

1.12 Chapter 6 presents the committee's conclusions and recommendations.

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6 *Journals of the Senate*, No. 75-9 February 2015, p. 2054.

7 On 25 March 2015, the reporting date was extended to 17 June 2015, *Journals of the Senate*, No. 89—25 March 2015, p. 2399; on 17 June 2015, the reporting date was extended to 4 August 2015, *Journals of the Senate*, No. 97—17 June 2015, p. 2686; on 4 August 2015 the reporting date was extended to 9 September 2015, *Journals of the Senate*, No103—10 August 2015, p. 2856; on 9 September 2015 the reporting date was extended to 15 September 2015, *Journals of the Senate*, No. 113—p. 3070; and on 15 September 2015 the reporting date was extended to 17 September 2015, *Journals of the Senate*, No. 116—p. 3120.

8 See: http://www.aph.gov.au/Parliamentary_Business/Committees/Senate/Community_Affairs.

The incidence of cancer in Australia

1.13 It is estimated that 45 780 people will die from cancer in 2015, an average of 125 deaths every day.[9] This figure represents approximately three out of every 10 deaths registered in Australia[10] and is 84 per cent higher than the number of deaths reported in 1982 (24 922 cases).[11]

1.14 The Australian Institute of Health and Welfare (AIHW) has estimated the risk of being diagnosed with cancer before the age of 85 is 1 in 2 for males and 1 in 3 for females.[12]

1.15 The number of expected diagnoses has increased 2.6 times compared to the number of new cancer cases reported in 1982 (47 417 cases). This corresponds to 467 cases per 100 000 people, compared to 383 cases per 100 000 people in 1982 (an increase of 22 per cent).[13]

1.16 The most common diagnoses for new cancer cases in 2014 was estimated to be:

- prostate cancer (17 050 cases);
- colorectal cancer (16 640 cases);
- breast cancer (15 410 cases);
- melanoma of the skin (12 640 cases); and
- lung cancer (11 580 cases).

1.17 Together, these forms of cancer comprise approximately 60 per cent of all expected diagnosed cancers.[14]

9 CDA, *Submission 53*, p. 1.

10 Australian Institute of Health and Welfare (AIHW) (2014), *Cancer in Australia, An overview 2014*, Cancer Series No. 90, Cat. no. CAN 88, Canberra: AIHW, p. 48.

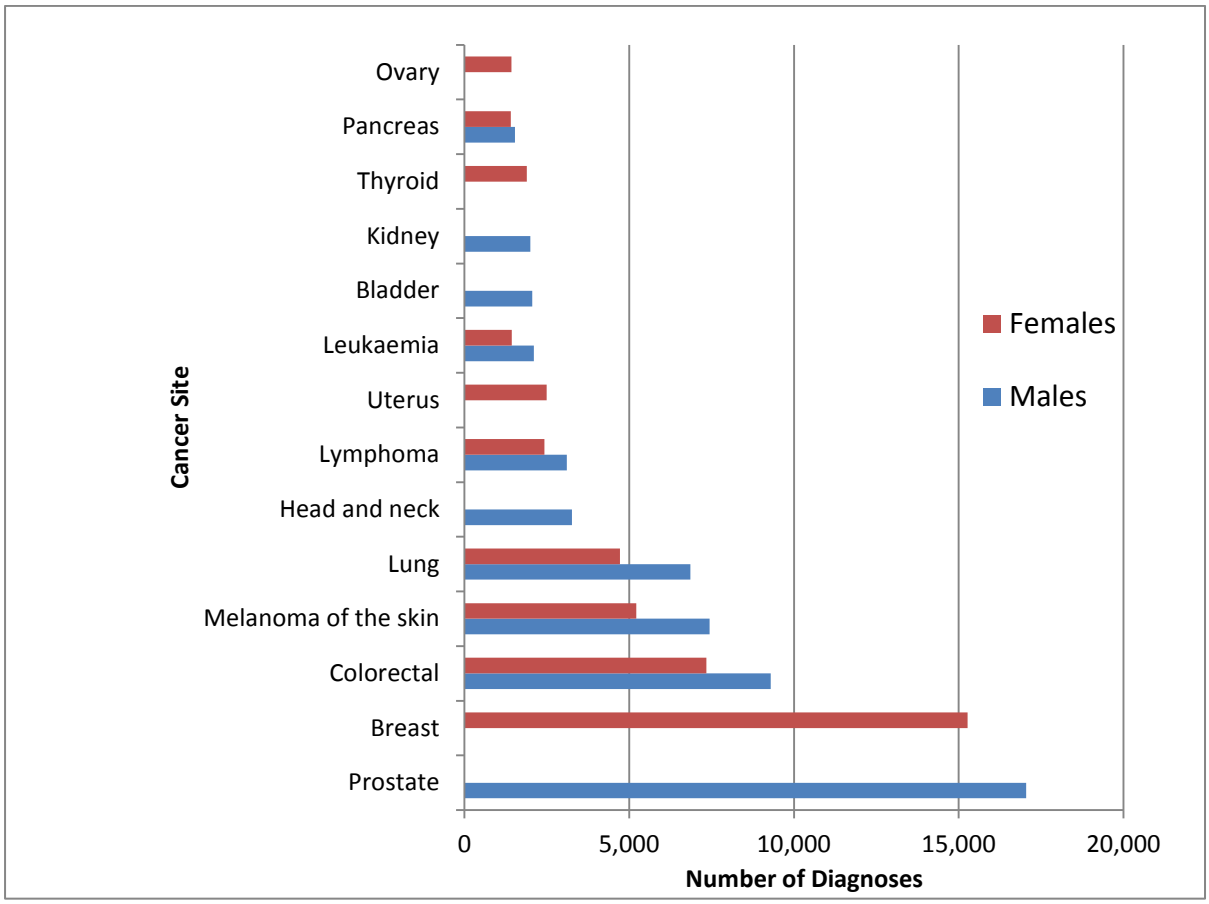
11 AIHW (2014), *Cancer in Australia, An overview 2014*, Cancer Series No. 90, Cat. no. CAN 88, Canberra: AIHW, p. 51. The increased number of deaths does not correspond to the number of deaths per 100,000 people: 168 in 2014 to 209 in 1982 (a decrease of 20 per cent).

12 AIHW (2014), *Cancer in Australia, An overview 2014*, Cancer Series No. 90, Cat. no. CAN 88, Canberra: AIHW, pp 15 and 17. These estimates do not include certain carcinomas which are not required to be notified to public health authorities.

13 AIHW (2014), *Cancer in Australia, An overview 2014*, Cancer Series No. 90, Cat. no. CAN 88, Canberra: AIHW, p. 19.

14 AIHW (2014), *Cancer in Australia, An overview 2014*, Cancer Series No. 90, Cat. no. CAN 88, Canberra: AIHW, p. 16.

Figure 1.1: Estimated 10 most common diagnoses of cancer, Australia, 2014



Australian Institute of Health and Welfare, *Cancer in Australia, An overview 2014*, Cancer Series No.90, Cat. No. CAN 88, Canberra: AIHW, p. 17.C

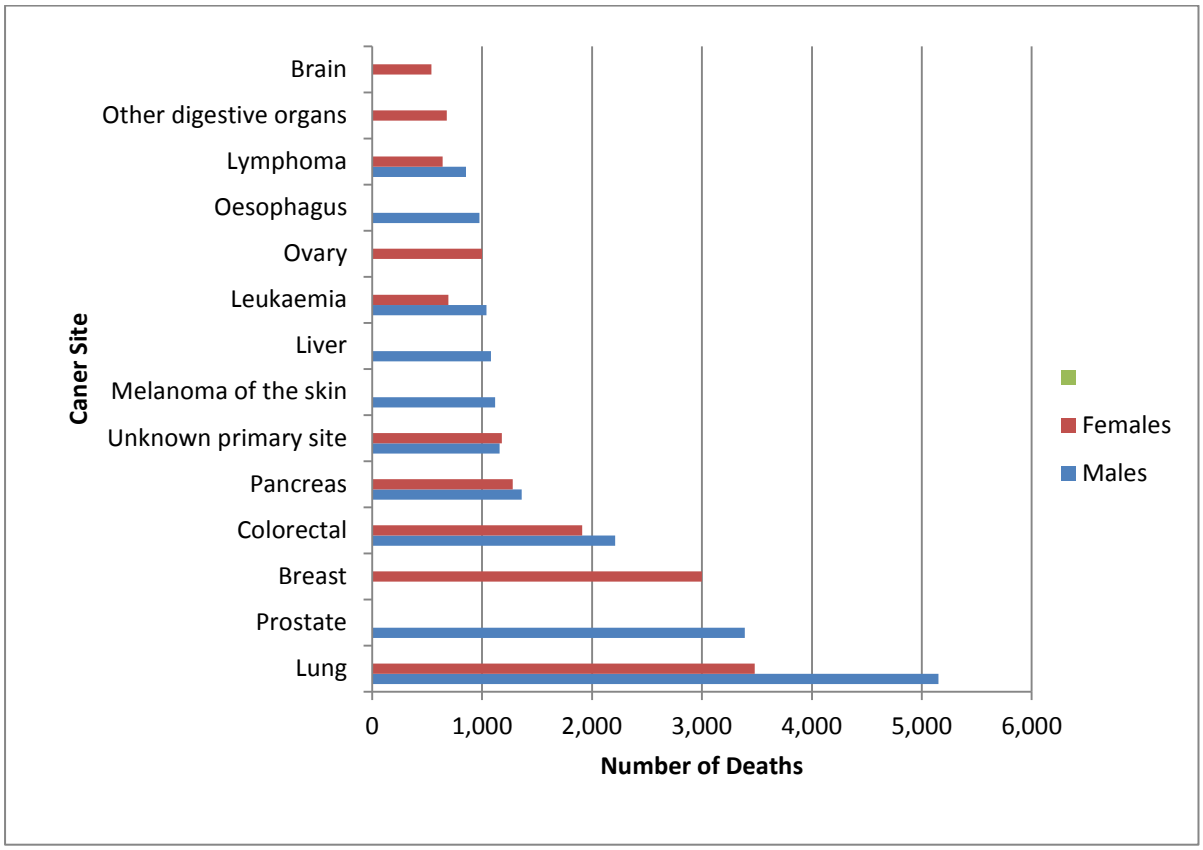
1.18 The AIHW predicted the diagnosis of 150 000 new cases by 2020, an increase of almost 40 per cent from 2007. The AIHW attributes this increase primarily to an ageing and increasing population, and has reported:

> **Which cancers will present the biggest burden in 2020?**
>
> For males, prostate cancer is expected to remain the most common cancer diagnosed in 2020 (25,300 cases), followed by bowel cancer and melanoma of the skin (about 10,800 cases each) and lung cancer (7,500 cases). For females, breast cancer is projected to continue to be the most common cancer diagnosed in 2020 (17,200 cases), followed by bowel cancer (9,200), melanoma (6,800) and lung cancer (6,100).
>
> **Which cancers are on the rise?**
>
> Age-standardised rates for liver cancer are projected to increase by 38% from 2007 to 2020 in males and 78% in females, while thyroid cancer rates are projected to increase by 33% in males and 62% in females.

Increases are also expected in rates for melanoma (30% males; 18% females), testicular cancer (25%) and lung cancer in females (16%).[15]

1.19 The most common causes of death resulting from cancer do not precisely correlate with the top five cancer diagnoses. Instead, lung cancer will be the most common cause of death (8 630 people), followed by colorectal cancer (4 120 people), prostate cancer (3 390 people), breast cancer (3 030 people) and pancreatic cancer (2 640 people). These five cancers represent just under half (48 per cent) of the total mortality from cancer, with lung cancer alone accounting for nearly one in five deaths (19 per cent).[16]

Figure 1.2: Estimated 10 most common causes of death from cancer, Australia, 2014



Australian Institute of Health and Welfare (2014), Cancer in Australia, An overview 2014, Cancer Series No. 90, Cat. no. CAN 88, Canberra: AIHW, p. 49.

15 AIHW (2012), *Cancer incidence projections, Australia 2011 to 2020*, Cancer Series No. 66, Cat. no. CAN 62, Canberra: AIHW, p. viii.

16 AIHW (2014), *Cancer in Australia, An overview 2014*, Cancer Series No. 90, Cat. no. CAN 88, Canberra: AIHW, p. 49.

1.20 The AIHW estimated that the risk of dying from cancer before the age of 75 years is one in nine for males and one in 13 for females. By the age of 85 years the risk increases to one in four for males and one in six for females.[17]

International comparison

1.21 The International Agency for Research on Cancer (IARC) (part of the World Health Organisation) maintains the GLOBOCAN database, which provides contemporary estimates on the incidence, mortality and prevalence of major cancer types at a national level for 184 countries.[18]

1.22 According to the most recent GLOBOCAN estimates, the number of new cancer cases diagnosed worldwide in 2012 was 14.1 million.[19] In that same year, 122,031 new cases were diagnosed in Australia, representing less than one per cent (0.87) of the global diagnoses. However, the incidence rate for cancer in Australia (323 per 100,000) was higher than the rate for other regions.[20]

1.23 In terms of mortality, the IARC estimated the number of deaths from cancer worldwide was 8.2 million in 2012. For Australia, 43,400 people were expected to die from cancer, a mortality rate of 96 per 100 000 people.[21]

Cancer as a national health priority

1.24 Cancer poses a complex challenge for the Australian healthcare system. Cancer is not one disease. It is many hundreds of diseases, each of which can manifest differently in each cancer patient. As the prevalence of cancer trends upwards, the health and economic impacts on individuals and the health system can be expected to continue to increase. At the same time, the costs of new cancer medicines are increasing at a faster rate than other new medicines.

1.25 Cancer is one of nine National Health Priority Areas (NHPA) and accounts for 19 per cent of the total disease-related burden, making it the highest disease-related burden on society.[22]

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17 AIHW (2014), *Cancer in Australia, An overview 2014*, Cancer Series No. 90, Cat. no. CAN 88, Canberra: AIHW, p. 50.

18 See: International Agency for Research on Cancer, World Health Organisation, *The GLOBOCAN Project*, http://globocan.iarc.fr/Default.aspx (accessed 23 January 2015).

19 See: International Agency for Research on Cancer, World Health Organisation, *All Cancers (excluding non-melanoma skin cancer): Estimated Incidence, Mortality and Prevalence Worldwide in 2012*, http://globocan.iarc.fr/Pages/fact_sheets_cancer.aspx (accessed 23 January 2015). This estimate did not include non-melanoma skin cancer.

20 See: International Agency for Research on Cancer, World Health Organisation, *Australia*, http://globocan.iarc.fr/Pages/fact_sheets_population.aspx?country=36 (accessed 23 January 2015).

21 See: International Agency for Research on Cancer, World Health Organisation, *Australia*, http://globocan.iarc.fr/Pages/fact_sheets_population.aspx (accessed 23 January 2015). The average world rate for 2012 was 102 per 100,000 people.

22 CDA, *Submission 53*, p. 2.

1.26 The annual cost of cancer to government has been placed between $4 billion and $5 billion per annum. This funding supports a range of measures along a continuum of care including: research, prevention programs and national screening programs as well as 'timely access to cost-effective, clinically indicated treatments through the Medicare Benefits Schedule (MBS) and the Pharmaceutical Benefits Scheme (PBS).' The Department of Health (DOH) states that the mix of funding must be balanced to deliver the best health outcome for the most cancer patients.[23]

1.27 Expenditure on cancer medicines accounts for one third of current cancer funding. As Figure 1.3 illustrates, in 2013-14, $1.5 billion was spent on subsidising the cost of PBS-listed cancer medicines.[24] This represents 16 per cent of the total PBS expenditure of $9.2 billion.[25]

Figure 1.3: Cost of PBS cancer medicines

PBS expenditure for cancer medicines	Benefits paid ($ billions)				
	2009-10	2010-11	2011-12	2012-13	2013-14
PBS and RPBS benefits paid - cancer	$0.994	$1.087	$1.135	$1.230	$1.486
Total PBS benefits paid – all medicines	$8.392	$8.873	$9.194	$8.996	$9.149

Department of Health, *Submission 197*, p. 22.

1.28 An additional $50 million is used to fund the Herceptin Program each year.[26]

Figure 1.4: Cost of Herceptin Program

Expenditure for Herceptin Program (non-PBS)	Benefits paid ($ millions)				
Financial year	2009-10	2010-11	2011-12	2012-13	2013-14
Total benefits paid	$48.9	$53.3	$54.1	$57.2	$53.3

Department of Health, *Submission 197*, p. 22.

1.29 DOH advised that cancer medicines are some of the most expensive medicines on the PBS:

> Despite reaching one sixth of total expenditure, cancer-related scripts (2.6 million) supplied in 2013-14 represent only around 1% of all PBS scripts (213.7 million). The funding benefited approximately 3% (over 337,250 patients) of the total 9.8 million patients supported through the PBS in that year.[27]

1.30 Cancer medicines are generally more expensive than non-cancer medicines and, as Figure 1.5 below illustrates, new cancer medicines make up an increasing proportion of total PBS expenditure on cancer medicines. DOH advised that:

23 DOH, *Submission 197*, p. 5.

24 *Submission 197*, p. 5

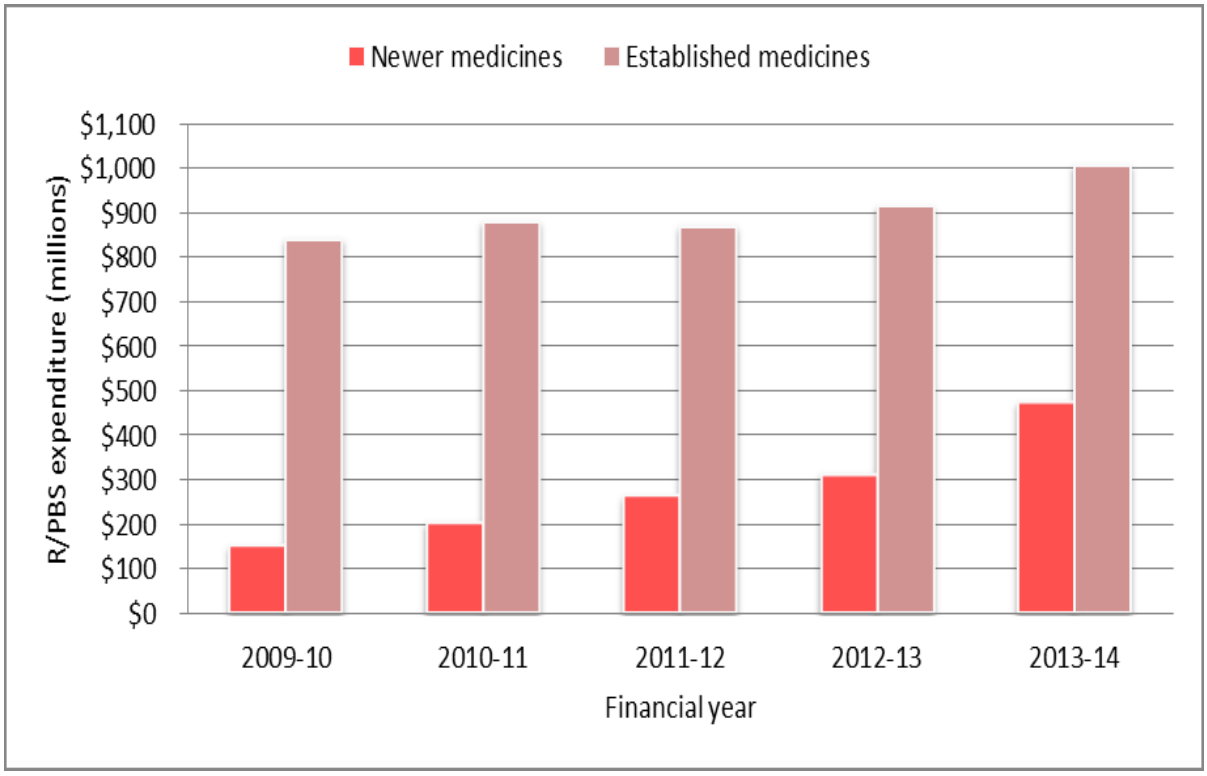
25 *Submission, 197*, p. 8.

26 *Submission 197*, p. 22. Herceptin is a treatment for breast cancer.

27 *Submission 197*, p. 8.

PBS benefits paid for newer cancer medicines increased at a rate of 33% per year over the last five financial years, compared to a growth rate of only 5% per year in benefits paid for established cancer medicines.[28]

Figure 1.5: Cost of established versus newer PBS cancer medicines



Department of Health, *Submission 197*, p. 23.

Assessment of cancer medicines in Australia

1.31 The Australian Government employs a range of processes and mechanisms to assess the quality, safety, efficacy, effectiveness and cost effectiveness of health technologies and procedures. Collectively, these processes and mechanisms are referred to as Health Technology Assessment (HTA).

The DOH advises that '[a] well-performing HTA system will:

- facilitate patient access to cost-effective health technologies that improve health outcomes;
- minimise the use of technologies that are ineffective or harmful;
- contribute to value for money investments in health technology in the context of limited health care resources;
- keep pace with evolving technologies, clinical practices and HTA methodologies;
- provide clear information on processes, rules and outcomes to stakeholders; and

28 *Submission 197*, p. 22.

- ensure the system is designed to achieve these outcomes in the most timely, effective, efficient and targeted way'.[29]

1.32 Concerns have been raised at the ability of the system to meet the above criteria and the vast majority of submissions have called for a fundamental review of the system.

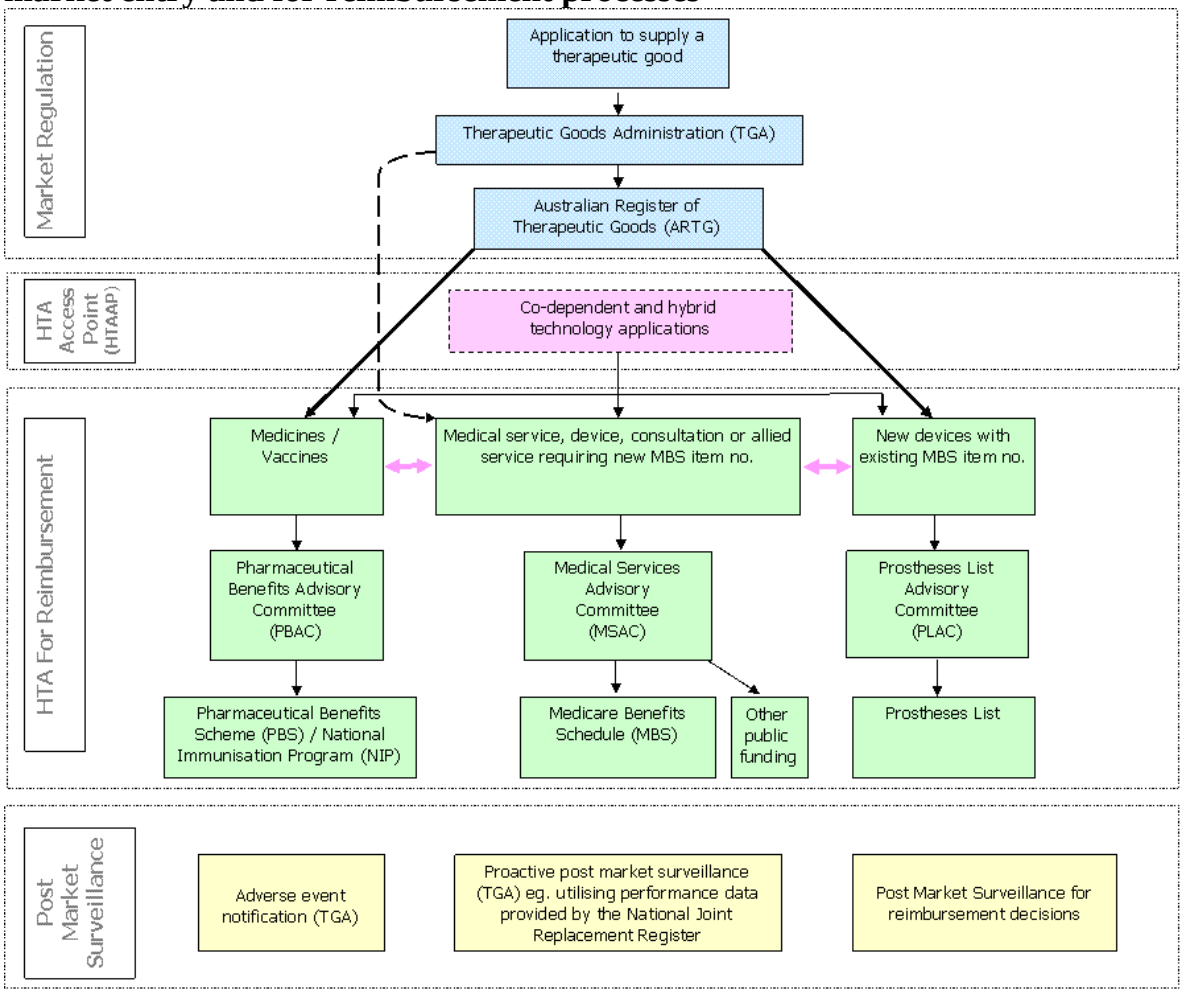
1.33 Through its HTA system, the Australian Government seeks to ensure the sustainability of the Australian Government's health financing arrangements. As Figure 1.6 illustrates, in order to gain approval and reimbursement of medicines in Australia sponsors are required to demonstrate the merit of the medicine against five critical requirements:

- quality, safety and efficacy, as assessed by the TGA;
- clinical and cost effectiveness, as assessed by the PBAC; and
- financial feasibility/acceptability as assessed by the Minister for Health and the Cabinet.[30]

29 DOH, Health Technology Assessment, What is Health Technology Assessment (HTA), http://www.health.gov.au/internet/hta/publishing.nsf/Content/about-1 (accessed 14 June 2015).

30 Deloitte Access Economics, Medicines Australia Oncology Industry Taskforce, 'Access to cancer medicines in Australia', July 2013, *Submission 142a*, p. v.

Figure 1.6: Map of current Australian Government HTA processes for market entry and for reimbursement processes



Department of Health, *Australian Government HTA Process*, Health Technology Assessment website.[31]

1.34 The following section provides an overview of the pathways through which cancer medicines are assessed, approved and reimbursed for use in Australia.

Therapeutic Goods Administration

1.35 Before a medicine can be made available to patients in Australia, it must first receive regulatory approval from the TGA. The TGA administers a uniform, national system of regulatory controls to ensure the quality, safety, efficacy and timely availability of therapeutic goods for human use. The TGA regulates therapeutic goods through:

- pre-market assessment;
- post-market monitoring and enforcement of standards; and

31 http://www.health.gov.au/internet/hta/publishing.nsf/Content/commonwealth-1 (accessed 14 June 2015)

- licensing of Australian manufacturers and verifying overseas manufacturers' compliance with the same standards as their Australian counterparts.[32]

1.36 The TGA approves and regulates products based on an assessment of risks against benefits, considering factors such as side effects, potential harm through prolonged use, toxicity and the seriousness of the medical condition for which the product is intended to be used.[33] While most therapeutic goods are required to undergo an evaluation by the TGA before they can be supplied in Australia, there are a number of ways that patients can gain access to products that have not been approved for use in Australia:

- Authorised prescribers: a medical practitioner may be granted authority to become an authorised prescriber of a specified unapproved therapeutic good to specific patients with a particular medical condition.
- Special access scheme: arrangements which provide for the import and/or supply of an unapproved therapeutic good for a single patient, on a case by case basis.
- Medicines that have not received TGA approval may be accessed only under specific circumstances.

1.37 Only medicines registered on the Australian Register of Therapeutic Goods can be included on the Schedule of Pharmaceutical Benefits (PBS Schedule).

The Pharmaceutical Benefits Scheme

1.38 Under the PBS the Commonwealth subsidises the cost of a wide range of prescription medications to all Australian residents who hold a medicare card.[34] Patients pay a contribution depending on their status as a general or concessional patient and the PBS provides safety nets, primarily through reimbursements paid to community or hospital pharmacies, to protect high medicine users from excessive medicine costs.[35]

1.39 The overarching framework for the operation of the PBS is provided in the National Medicines Policy (NMP). Among other things, the NMP provides for 'timely access to the medicines that Australians need, at a cost individuals and the community can afford'.[36] The PBS Schedule lists all medicines available to be dispensed to patients at a Government-subsidised price.

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32 DOH, Therapeutic Goods Administration, How the TGA regulates, https://www.tga.gov.au/how-tga-regulates (accessed 7 June 2015).

33 DOH, Therapeutic Goods Administration, How the TGA regulates, https://www.tga.gov.au/how-tga-regulates (accessed 7 June 2015).

34 Department of Parliamentary Services, Parliamentary Library, *Growth in expenditure on high cost drugs in Australia*, Research Paper Series, 2014-15, 7 January 2015,

35 DOH, *Submission 197*, p. 7.

36 DOH, *National Medicines Policy Document*, 2000, p. 1.

1.40 On 27 May 2015, the Minister for Health, the Hon Sussan Ley MP, announced a package of reforms to the PBS. In introducing the Pharmaceutical Benefits Scheme Access and Sustainability Package (reforms), the Minister stated:

> This reform package is designed to be a sensible start that focuses on longer-term structural reform to enable ongoing investment in new medicines while ensuring they remain affordable for patients and taxpayers.[37]

1.41 The reforms include:

> …a five per cent reduction in the price taxpayers pay for on-patent medicines that have been listed for five years or more on the PBS. This is expected to deliver efficiencies of about $1 billion to ensure new F1 medicines can be listed for patients as well.[38]

1.42 The committee notes that the potential for this measure to impact on research and development of new medicines was raised during consideration of the National Health Amendment (Pharmaceutical Benefits) Bill 2015.[39]

The Pharmaceutical Benefits Advisory Committee

1.43 The PBAC is an independent expert body comprised of doctors, health professionals and consumer representatives appointed by the Australian Government. The PBAC meets three times a year to consider new medicines for listing on the PBS. No new medicine can be listed unless the PBAC makes a positive recommendation.

1.44 When recommending a medicine for listing, the PBAC takes into account the medical conditions for which the medicine was registered for use in Australia, its clinical effectiveness, safety and cost-effectiveness. The PBAC is assisted in its analysis and advice by the Drug Utilisation Sub Committee and the Economics Sub Committee.[40]

1.45 Following a positive recommendation from the PBAC, the sponsor of the medicine is required to negotiate pricing and any applicable prescribing restrictions with the DOH.[41] If the cost is more than $20 million in any one year of the Forward

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37 The Hon Sussan Ley MP, 'Pharmaceutical Benefits Scheme to be reformed', 27 May 2015, https://www.health.gov.au/internet/ministers/publishing.nsf/Content/health-mediarel-yr2015-ley063.htm (accessed 16 September 2015).

38 The Hon Sussan Ley MP, 'Pharmaceutical Benefits Scheme to be reformed', 27 May 2015, https://www.health.gov.au/internet/ministers/publishing.nsf/Content/health-mediarel-yr2015-ley063.htm (accessed 16 September 2015).

39 Senator Hon Jan McLucas, *Senate Hansard*, 23 June 2015, p. 4159.

40 DOH, *Pharmaceutical Benefits Advisory Committee (PBAC)*, http://www.pbs.gov.au/info/industry/listing/participants/pbac (accessed 30 May 2015).

41 Prior to the 2014-15 Budget, pricing of pharmaceuticals was managed by the Pharmaceutical Benefits Pricing Authority (PBPA). The abolition of the PBPA was expected to help streamline the PBS listing process.

Estimates, the recommendation must then be approved by the Minister for Health or Cabinet.[42]

Medical Services Advisory Committee (MSAC)

1.46 A separate but similar process applies for the assessment of medical services or technology. The MSAC is an independent expert committee that provides advice to the Minister for Health on the strength of evidence relating to the comparative safety, clinical effectiveness and cost-effectiveness of any new or existing medical service or technology, and the circumstances under which public funding should be supported through listing the service and technology on the MBS. The MSAC meets up to four times a year.

1.47 Co-dependent and hybrid pharmaceuticals are currently considered separately by the PBAC and the MSAC using different approaches to assessing evidence against the HTA criteria. This is because listing needs to occur under two separate funding programs.

Alternate access schemes

Life Saving Drugs Programme (LSDP)

1.48 The Australian Government provides subsidies for a limited range of medicines not eligible for funding under the PBS through the LSDP.[43] Through the LSDP, eligible patients are able to gain access to expensive lifesaving drugs for very rare life-threatening conditions. The LSDP currently subsidises ten medicines for eligible patients with one of seven rare and life threatening diseases.

1.49 Submissions for a drug to be considered for inclusion in the LSDP must be lodged in conjunction with submissions to the PBAC for PBS listing. Submissions are received in March, July and November each year by DOH. If the PBAC accepts that a drug is clinically effective for the proposed indication but rejects it for listing on the PBS on the grounds that it is not cost effective, the sponsor of the drug may request the application be considered for inclusion in the LSDP.[44]

1.50 In April 2014, the then Minister for Health, the Hon Peter Dutton MP, announced a post-market review of the LSDP to examine issues such as access and equity, value for money and the future administration of the program.[45]

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42 DOH, *Submission 197*, p. 13.

43 Subsidised access is provided to eligible patients under section 32B of the *Financial Framework (Supplementary Powers) Act 1997* and schedule 1AA of the Financial Framework (Supplementary Powers) Regulations 1997, item number 415.009 (Targeted Assistance – Pharmaceuticals). See DOH, Life Saving Drugs Programme, Post Market Review, *Issues Paper*, April 2015, p. 7.

44 DOH, Life Saving Drugs Programme (LSDP), Post Market Review, *Issues Paper*, April 2015, p. 7.

45 DOH, LSDP, Post Market Review, *Issues Paper*, April 2015.

Orphan Drugs Program (ODP)

1.51 The ODP, administered by the TGA, was established to encourage drug manufacturers to develop and market medicines affecting small populations. An orphan drug is a medicine that is intended to treat, prevent or diagnose a rare disease, or is not commercially viable to supply to treat, prevent or diagnose another disease or condition.

1.52 Before an application to register an orphan drug is made, a sponsor must seek orphan drug designation. The quality, efficacy and safety of orphan drugs are assessed at the same standard as other registered medicines. Orphan drug designation by the TGA does not mean that the drug will be automatically considered for inclusion in the LSDP.[46]

Comparable international models

1.53 A number of submissions highlighted models introduced overseas to improve access to new cancer drugs, and involve patients in the evaluation process.

United Kingdom

1.54 In 2010, the United Kingdom established the Cancer Drugs Fund to assist patients to access certain drugs before they receive National Institute for Health and Care Excellence (NICE) approval.[47] According to a 2013 report by Deloitte Access Economics, the fund subsidises drug treatments, including radiopharmaceuticals, for patients who have been unable to access a drug recommended by their oncologist.[48] The Cancer Drugs Alliance noted in its submission that the fund:

> continues to cover approximately 59 cancer drugs and during the 5 years it has been in existence has allowed more than 60 000 cancer patients to receive treatment they would have not have otherwise had access to.[49]

1.55 Patients in the UK can also be involved in setting decision-making criteria for the approval of new drugs and can participate in the Health Technology Assessment (HTA) committee.[50]

Canada

1.56 In Canada, the pan-Canadian Oncology Drug Review (pCODR) was established in 2007 separate to the Common Drug Review (CDR) to assess cancer drugs and make recommendations to provincial cancer agencies/governments to guide

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46 DOH, Orphan drugs, https://www.tga.gov.au/orphan-drugs, (accessed 1 June 2015)

47 Medicines Australia (MA), *Submission 142*, p. 22.

48 Deloitte Access Economics, Medicines Australia Oncology Industry Taskforce, 'Access to cancer medicines in Australia', July 2013, *Submission 142a*, p. 49.

49 Cancer Drugs Alliance, *Submission 53*, p. 4.

50 MA, *Supplementary Submission 142*, p. 8.

drug funding decisions. In April 2014, pCODR was integrated into the Canadian Agency for Drug Technologies and Health (CADTH).[51]

1.57 As part of the pCODR, patients can provide input at the beginning of and throughout the process for evaluating new cancer drugs.[52] Medicines Australia noted that the pCODR model 'reflected a deliberate decision to adopt a stakeholder focussed approach with cancer and to overcome challenges faced in HTA'.[53]

United States of America

1.58 The United States (US) Food and Drug Administration (FDA) regulates the use of prescription medications in the US. The FDA provides pharmaceutical companies with four pathways that 'get important new drugs to the patient earlier' to 'treat serious conditions and fill an unmet medical need'.[54] These are aimed at:

- Expediting Product Development through:
    - Fast Track Designation
    - Breakthrough Therapy Designation
- Expediting Registration through:
    - Accelerated Approval
    - Priority Review.[55]

1.59 Fast Track Designation works by facilitating the development and expediting the review of medications. A pharmaceutical company applies for fast track consideration when there is no therapy available or if 'a therapy may be potentially better than available therapy'.[56]

1.60 Merck Sharp and Dohme describe the Breakthrough Therapy Designation as 'unique in that the FDA invests significant resources and time in numerous discussions with the sponsor and close co-operation in the development of the clinical program'.[57] Depending on the type of application and the stage of development, an application to one of the four pathways can result in a range of different assistance options including

51 Medicines Australia (MA), *Submission 142*, p. 22–23.

52 MA, *Submission 142*, *Attachment 2*, p. 9.

53 MA, Submission 142, p. 22.

54 United States Food and Drug Administration, *Fast Track*, September 2015, http://www.fda.gov/forpatients/approvals/fast/ucm405399.htm (accessed 16 September 2015).

55 Merck Sharp and Dohme (MSD), *Submission 120a*, p. 4.

56 United States Food and Drug Administration, *Fast Track*, September 2015, http://www.fda.gov/forpatients/approvals/fast/ucm405399.htm (accessed 16 September 2015).

57 MSD, *Submission 120a*, p. 2.

access to rolling review, access between pathways and increased access to FDA advice during the approvals process.[58]

58 United States Food and Drug Administration, *Breakthrough Therapy*, September 2015, http://www.fda.gov/ForPatients/Approvals/Fast/ucm405397.htm (accessed 16 September 2015).