Chapter 4

Impact of delayed access to cancer medicines on cancer patients

4.1 Throughout the inquiry the committee heard that timely, affordable access to treatments and quality care are inherently linked for cancer patients. A strong message in the submissions was that most cancer patients, particularly those with advanced disease, cannot afford to wait for effective treatment options to become available in a subsidised form in Australia.

4.2 The committee heard that the quality of cancer care is negatively impacted when the appropriate course of treatment for a patient's cancer profile is either not available in Australia or is not subsidised via the Pharmaceutical Benefits Scheme (PBS).[1]

4.3 The committee received evidence that suggested underuse of potentially successful treatments has played a part in the low survival rates of patients with lung, colorectal and ovarian cancers in Denmark and the United Kingdom.[2]

4.4 This chapter examines the impact difficulties in accessing cancer medicines can have on the quality of care available to cancer patients. It considers the avenues available to individual cancer patients to access cancer medicines that have not been listed on the PBS before considering the particular challenges faced by rural and regional cancer patients, patients with rare and less common cancers and children, adolescents and young adults.

The impact of delayed access on cancer patients and their families

4.5 The committee received many submissions from individual cancer patients and their families urging the committee to support the expedited listing of specific cancer medicines. Personal accounts from cancer patients and their families highlighted the distress experienced by patients and their families when apparently superior treatment options are not available in Australia via the PBS. The committee heard that delays in the approval of cancer treatments, together with delays in the commencement of treatment due to the need to seek special approval or organise finance is a source of significant stress for cancer patients.[3] The committee heard repeatedly that cancer patients with advanced disease cannot afford to wait and that the knowledge that a particular drug may enable them to gain more valuable time or

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1 Cancer Voices Australia, *Submission 49*, p. 5.

2 De Angelis R et al, Cancer survival in Europe 1999-2007 by country and age: results of EUROCARE 5—a population-based study. Lancet Oncol 2014; 15:23-34, cited by Novartis Oncology, *Submission 87*, p. 28.

3 Pharmaceutical Society of Australia, *Submission 176*, p. 6.

improve their quality of life, but at a price outside their ability to pay, is a source of great anguish.[4]

4.6 Ms Lorraine Hoskin's account of the plight of a young family friend is indicative of a great many submissions to the inquiry:

> A young family friend has recently been diagnosed with a rare form of cancer. After the chemotherapy options have now been exhausted without a positive result his only option to gain more valuable time is a very expensive cancer drug. The decision whether to start this treatment is a major stress to him because of the financial cost which will affect the future of his young wife and family.
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> On top of the extreme suffering he has endured fighting this wretched disease, the added torment of knowing there is a drug which could help him, but at a huge cost is very unfair.[5]

4.7 Submitters described the significance to cancer patients and their families of maintaining a normal life. Many described the physical, emotional and mental toll associated with cancer treatments that disrupt everyday activities and separate cancer patients from family life. Cancer patients spoke of the importance of being able to return to the workforce and contribute to society, and emphasised the distress felt when this is not possible. Ms Lee McKerracher told the committee:

> The cost of treatment may be expensive, but how can you put a price on giving a family member an extra 6 months, 12 months, 2 years with their family. Some may even be able to return to the workforce or their studies to contribute to society with whatever time they have left – we should give them the chance to do so.[6]

4.8 Evidence to the committee emphasised the potential of many new cancer medicines to improve the quality of life of many cancer patients by reducing the need for costly and invasive treatments such as surgery or hospitalisation. For example, Medicines Australia (MA) noted that consumer comments received by sponsors indicate that a simple and short outpatient administration of a single new targeted cancer drug has greater benefit compared to a treatment regimen involving multiple chemotherapies, long infusion times and the requirement to travel to the hospital several times per week or month.[7]

4.9 For Ms Karen Cowley, a breast cancer survivor, access to the drug Kadcyla has stabilised her disease and allowed her to feel a degree of control in her life:

> Living with cancer affects a person physically, emotionally, and mentally. It can interfere with everyday activities. For many years I felt I was on a tightrope having many different regimes of drugs to manage my disease with many side effects. Kadcyla has been a revelation, since May 2014, I

4 See for example: Mr Paul Hobson, *Submission 6*, p. 2.

5 *Submission 30*, p. [1].

6 *Submission 25*, p. [1].

7 Medicines Australia, *Answer to question on notice*, 20 April 2015 (received 25 May 2015), p. 3.

> feel that I have some control in my life as my disease is stable and I have less side effects. Administration of the drug is also less invasive, with only one infusion taking less than an hour whereas other medications took several hours.[8]

4.10 Such benefits extend beyond the patient to those supporting them who are spared months of worry and stress and caregiver time.[9]

4.11 The committee received many submissions from cancer patients and their families highlighting the significant physical, financial and psychological impacts of being unable to access new and innovative cancer drugs. Many patients are faced with the harsh dilemma of either paying a significant amount of money to access the latest, most effective treatments for their cancer or being unable to access the treatment at all.[10] Ms Louise Marshall of the Australian Melanoma Consumer Alliance told the committee that many cancer patients are faced with the choice of selling their home, accessing their super or relying on the fundraising efforts of friends and family.[11]

4.12 Bowel Cancer Australia told the committee:

> The alternative for many is forgoing treatment and therefore the possibility of precious extra time with their loved ones.[12]

4.13 A number of submitters gave accounts of their frustration at finding the medicine needed to treat their cancer was not listed on the PBS for their cancer, or for their stage of the disease. Ms Bridget Whelan, who has ovarian cancer, told the committee that:

> I finished chemotherapy in May 2014 and spent some time recovering and getting my health back. Chemo takes quite a toll on your body and your mind.
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> Since then I have been on what is called a "maintenance drug" – it's a drug to slow tumour growth and prolong your remission. I pay for this drug. When I started it, it wasn't on the PBS at all. It now is, but not for me. Only for women diagnosed for the first time. So I entered into a co-pay arrangement with the pharmaceutical company. It's expensive. It's more than another mortgage. I am so lucky I am in a position to do this. I imagine only a handful of women in Australia can and that disturbs me greatly. This is a drug which has shown good results in worldwide trials in prolonging the remission of women just like me. The alternative, the standard Australian Government funded approach, is that after my last chemo, I would do nothing and just wait for it to come back.[13]

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8 Ms Karen Cowley, *Submission 11*, p. 1.

9 Medicines Australia, *Answer to question on notice*, 20 April 2015 (received 25 May 2015), p. 3.

10 Cancer Drugs Alliance (CDA), *Improving Access to Cancer Medicines*, White Paper, March 2015, p. 8.

11 *Committee Hansard*, p. 61.

12 Bowel Cancer Australia, *Submission 149*, p. 6.

13 Ovarian Cancer Australia, *Submission 137*, p. [11].

4.14 Mr Scott Beyer, who has non-Hodgkin lymphoma, told the committee of the disappointment of knowing the preferred treatment for his cancer is not listed on the PBS:

> When we first met with the head oncologist at the Alfred hospital to discuss my options he said I would be better off to stay on Bentuximab for an extended period of time or until the drug possibly failed to keep the cancer under control. We alluded to the fact that it was going to be such financial burden on our family that this wasn't going to be possible. He was of the understanding that this drug was on the PBS, but what he didn't realise was that it didn't cover my type of cancer. The fact that he believed I would be better suited staying on Brentuximab for an extended period of time in lieu of going through an arduous and sometimes fatal procedure shows how important the access to these drugs is, but the fact of the matter is it is financially out of my reach.
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> In this day and age this is just unacceptable and truly disappointing.

4.15 With the support of family and friends, Mr Beyer has been able to raise funds to support his treatment, but expressed concern that 'this generosity can't last forever'.[14]

4.16 Submitters expressed concern that Australian patients appear to be missing out on new cancer treatments and are relying on older alternative treatments, with harsh side effects, compared with cancer patients in many other countries.[15] Mr Janis Kinne told the committee of the constant stress of living with advanced prostate cancer and not being able to access best practice treatments available in other countries:

> It is very tense and very stressful for anyone afflicted and their families. Hormone resistant cancer can appear at any time for no evident reason. When it does the prognosis is not good and life expectancy starts down the slippery slope. Men with prostate cancer in United States are able to be treated successfully with Zytiga or Xtandi before chemotherapy. It is frustrating that Zytiga and Xtandi are not available in Australia on the Pharmaceutical Benefits Scheme before chemotherapy. Instead of getting best practice treatment, I have to undergo chemotherapy with its harsh side effects before I can get access to Zytiga and Xtandi on the Pharmaceutical Benefits Scheme.[16]

Impact on medical advice

4.17 Delays in access to subsidised medicines also pose challenges for medical practitioners. The committee heard that cancer physicians often encounter the moral and ethical dilemma of raising the potential of treatment with particular cancer

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14 Mr Scott Beyer, *Submission 104*, p. 1.

15 See for example: Mr Andrew Warden, *Submission 7*, p. 5; Mr David Prestridge, *Submission 5* [1]; Ms Natalie Cook,

16 *Submission 12*, p. [1].

medicine with a patient who may not be able to access that medicine in a subsidised form in Australia.[17]

4.18 Breast Cancer Network Australia (BCNA) told the committee that past research indicates that women have not always been told about expensive new treatments if the medical oncologist thought the patient could not afford it, for fear of distressing the patient and her family.[18] Rare Cancers Australia (RCA) raised a similar concern:

> … where a drug is not funded for the particular indication that afflicts the patient, the clinician is faced with less optimal choices, namely:
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> - Prescribe a "second choice" medication that is funded through the PBS.
> - Seek compassionate or charitable access to the first choice medicine through a compassionate program or clinical trial. This option is not always available.
> - Present the facts to the patient and let them decide if they can fund their own treatment.
> - Deliberately misdiagnose the patient so that the patient can access the treatment through the PBS for a funded indication. **In these circumstances we are confronting clinicians with the choice of fraud or inadequate care.**[19]

4.19 Mr Anthony Steele, Head of Blood Cancer Support, Leukaemia Foundation of Australia (LFA) said:

> Patients are not being told about all available therapies. We think there should be some sort of onus on health professionals to provide information on all available therapies. We get stories from patients who are told that to access a therapy they need to pay for it. They have been prepared to sell their house and been very devastated by it. In consultation with the Leukaemia Foundation we have put them through other means of getting access to the therapies, sometimes free of charge or highly discounted. It was at maybe a competing hospital or a different centre, across the river or in a different state. They are not advised of all available therapies. We think patients should be advised of all available therapies.[20]

4.20 Submitters argued that a cancer patient should have the choice of trying new and innovative treatments, particularly if these have the potential to offer an improved quality of life. Ms Jolanda Visser told the committee that she would like to have the option of accessing drugs that may improve her quality of life or slow the progression of her disease:

> I respect the system and also understand that the government does not want to make drugs available without [them] being thoroughly tested. However, I also think that we patients should be given a choice. It should be my choice

17 CDA White Paper, p. 8.

18 *Submission 90*, p. 6.

19 *Submission 92*, p. [8].

20 *Committee Hansard*, p. 9.

> to try other drugs, as if I am forced to continue to take the medication I am currently taking, I will also have to face some realities of a diminished quality of life, progression in my disease which could introduce new risks and even a shorter life span.[21]

Alternative pathways for access to cancer drugs

4.21 Cancer patients have a limited number of options available to them to gain special or off- label access to cancer medicines that are not available through the PBS. Cancer patients may receive off-label access via:

- compassionate access programs, where a pharmaceutical company may provide patients with access to new medicines, often free of charge;
- hospital formularies, where the hospital agrees to pay for the treatment for that individual patient;
- clinical trials; or
- by meeting the associated costs out of private funds.

4.22 For cancer medicines that are not listed on the Australian Register of Therapeutic Goods, the Therapeutic Goods Administration (TGA) manages the Special Access Scheme (SAS). The SAS provides for the import and/or supply of an unapproved therapeutic good for a single patient, on a case-by case basis.[22]

4.23 The term off-label refers to the use of a medicine in ways other than specified in the TGA approved product information. Off-label use includes when a medicine is prescribed or administered:

- for another indication;
- at a different dose;
- via an alternate route of administration; or
- for a patient or an age or gender outside the registered use.

4.24 The committee notes that off-label prescribing is an integral part of patient care for many cancer patients. The Council of Australian Therapeutic Advisory Groups has stated:

> In some circumstances, off-label use of a medicine may represent the best available option for a patient or the standard of care. The off-label use of medicines allows patients to access innovative and potentially useful new medicines or older medicines for new indications, dose or routes based on recent evidence. In patient groups, such as paediatrics, oncology, psychiatry

21 *Submission 139*, p. 2.

22 Department of Health, *Therapeutic Goods Administration*, Special access scheme, www.tga.gov.au (accessed 15 September 2015)

> and palliative care, off-label use of medicines is prevalent and may provide the only treatment option.[23]

4.25 Off-label prescribing is common in paediatric oncology and is not limited to new medicines. The Australian and New Zealand Children's Haematology/Oncology Group (ANZCHOG) submitted:

> A recent study at the Sydney Children's Hospital showed that 68% of standard chemotherapy agents were prescribed "off label". During the same time period over 80% of "new" anti-cancer therapies were prescribed "off label" (personal communication). The extent of "off label" prescribing reflects the fact that the levels of evidence required by regulatory authorities are not available for rare diseases such as paediatric cancer.[24]

4.26 The committee notes that both patients and medical practitioners face a level of risk in the use of off-label medicines as there are clinical, safety, ethical, legal and financial issues associated with such use which require careful consideration.[25] Off-label medicines may have less supporting evidence and have undergone less scrutiny for efficacy, safety and cost-effectiveness than medicines registered by the TGA. Prescribers are therefore expected to exercise professional judgement in prescribing off-label use of medicines.[26]

4.27 At the same time, the committee notes evidence that some cancer patients have a higher propensity for the risks associated with such use. Mr Barrie Littlefield, Head of Engagement, Cure Brain Cancer Foundation (CBCF) told the committee:

> It is fair to say that it is something that is very often overlooked by the medical community and others. In the vast majority of cases, people living with brain cancer have a very short time to live. Therefore, their risk propensity is extremely high in many cases—far higher than their doctors sometimes realise. They are quite prepared to take extreme risks when they need to if they think they have hope of a cure. So I think there needs to be a resetting around the ethical boundaries that are currently being set around access as well. I think it is well worth considering that.[27]

Compassionate or early access pathways

4.28 Compassionate and early access programs are initiated by the sponsors of a medicine and approved by the drugs or therapeutics committees of participating

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23 Council of Australian Therapeutic Advisory Groups, Rethinking medicines decision-making in Australian Hospitals, Guiding Principles for the quality use of off-label medicines, November 2013, p. 6.

24 *Submission 152*, p. 7.

25 Council of Australian Therapeutic Advisory Groups, Rethinking medicines decision-making in Australian Hospitals, Guiding Principles for the quality use of off-label medicines, November 2013, p. 4; Department of Health, *Submission 197*, p. 20.

26 Council of Australian Therapeutic Advisory Groups, Rethinking medicines decision-making in Australian Hospitals, Guiding Principles for the quality use of off-label medicines, November 2013, p. 6.

27 *Committee Hansard*, p. 15.

hospitals.[28] Most compassionate access programs provide access for a limited time or to a pre-specified financial commitment. MA told the committee that in more than two thirds of cases this access is used to cover the gap between TGA registration and PBS reimbursement.[29] MA referred the committee to research undertaken by Deloitte Access Economics (DAE), which found that nearly 5000 patients were provided with compassionate access in Australia during 2011-12 from a sample of nine pharmaceutical companies. In most cases the access was provided free of charge.[30] The DAE report states that approximately $10 million of cancer medicines are provided to patients prior to PBS listing, or even experimentally prior to TGA approval, through specialist cancer centres.[31]

4.29 However, MA noted that the sustainability of such programs is a significant issue for the pharmaceutical industry, particularly when there are delays in achieving PBS listing:

> …companies are frequently criticised by clinicians, the Government and PBAC when unanticipated, lengthy delays in listing decisions mean that ongoing access cannot be commercially sustained indefinitely.[32]

4.30 Mr Timothy James, Chief Executive Officer, MA, told the committee:

> …we support our members in their efforts to provide access to new medicines to patients, but we point out that these are not themselves sustainable access models and should not be performing the role of the PBS.[33]

4.31 Key concerns raised in relation to compassionate access programs are that cancer patients are reliant on their treating clinician to lobby for them to gain compassionate access to treatment and that such access is not assured.[34]

4.32 Ovarian Cancer Australia (OCA) noted that access to compassionate access program is often subject to strict eligibility criteria, and that even if a patient is successful in gaining entry, many patients still face significant costs:

> While compassionate access schemes do sometimes exist in the interim period between TGA approval and PBS listing, these are at the discretion of the drug sponsor and they do not necessarily cover all of the drug costs. We have seen many instances of patients facing considerable financial burden to meet the costs of non-PBS listed medicines.[35]

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28 MA, *Submission* 142, Appendix C, Deloitte Access Economics, Access to cancer medicines in Australia, Medicines Australia Oncology Industry Taskforce, July 2013, p. 51

29 *Submission 142*, p. 25.

30 *Submission 142*, p. 25.

31 *Submission 142*, p. 25.

32 *Submission 142*, p. 26.

33 *Committee Hansard*, p. 2.

34 Melanoma Patients Australia, *Submission 116*, p. [2].

35 *Submission 137*, p. 4.

4.33 The committee heard a number of personal accounts indicating the magnitude of the financial burden faced by cancer patients. Mrs Karen Cowley described for the committee the personal impact of making a significant contribution to receive the drug Kadcyla[36] through a pharmaceutical company's compassionate program:

> I paid $15,000. I am now on a disability pension and my husband has just turned 60 and working 6 days a week to make ends meet. It's too much for him. Going forward we are unable to fund another drug. Even though I am a skilled professional, I am trying to get part-time work to make ends meet but being in my late 50s with cancer, difficult to find work. The irony is that Kadcyla has made me well enough to feel I can work again. I live in hope, as it goes against my grain to be a pensioner which has been my last option as our savings are depleted due to my loss of income and 8 years of out of pocket medical costs.[37]

4.34 In some cases, cancer patients must reach a threshold of treatments before they become eligible for a compassionate program. Mr Chris Brugger, who is currently taking the drug brentuximab vedotin to treat Hodgkin's lymphoma, told the committee that his treatment costs his family $16 000 per dose every three weeks. In the event that Mr Brugger requires more than nine doses, the pharmaceutical company will supply the drug free of charge:

> The drug company has a compassionate program where, once you get to nine doses, they will supply it after that. There is research on this drug, in America, that shows it used to be capped at 16 doses but now you can stay on it indefinitely, as maintenance. My oncology nurses have spoken to the drug reps and they have said the compassionate program will be fine for me. If I need to get over nine doses, they said I am a perfect candidate for it, because I am young and otherwise healthy. I am a perfect candidate for it. If I were in my 60s or something it probably would not be as good a prospect.[38]

4.35 Mrs Lesley Royle's account of her access to the drug Agrylin on compassionate grounds prior to it being added to the PBS highlights the uncertainty surrounding a patient's ability to secure private health insurance coverage to defray out-of-pocket costs:

> My Private Health Cover agreed to pay the difference between the private script price (which was $75.00 at the time) and the actually cost of the drug. I was prescribed 3 months' supply and my health fund pulled their funding, leaving me and my husband to pay nearly $2000.00 in pharmaceutical costs.[39]

4.36 The committee heard that there has not been a systematic effort to gauge the ability and willingness of health funds to fund non-listed PBS items. The DAE report

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36 Kadcyla is a treatment for HER2-positive metastatic breast cancer

37 Ms Karen Cowley, *Submission 11*, p. 1.

38 *Committee Hansard*, p. 19.

39 Mrs Lesley Royle, *Submission 113*, p. 2.

noted that health fund payments appear predominately to be *ex gratia* and legislative requirements surrounding the coverage of non-PBS listed medicines are unclear.[40]

Public hospital formulary

4.37 The committee also notes concerns raised about the availability of cancer medicines through public hospital formularies. Access to subsidised medicines for admitted public patients in public hospitals is dependent on the formulary of individual hospitals and in Queensland, the state-based formulary. The decision to list pharmaceuticals on the formulary of Australian hospitals is a consideration for the drug committees of individual hospitals or states and territories.[41]

4.38 The committee heard that as there is no single streamlined process across institutions and jurisdictions to assess proposed formulary listing of a medicine, the timeframe of each listing process is variable.[42] Requests to prescribe drugs outside a hospital's list of approved medications, such as new anti-cancer therapies, usually involves an application to the hospital executive or jurisdictional advisory body. [43] CanTeen told the committee that varying policies between hospitals and states can lead to inequities in access:

> While clinicians can request that their hospital pay for such drugs via individual patient usage applications, most public hospitals also cannot afford such drugs. Moreover, while one hospital may approve an individual patient usage application for a specific agent, another hospital may not, thereby creating further inequity of access.[44]

4.39 Link HealthCare submitted that funding of cancer medicines through the hospital system is limited for both in-patient and out-patient medicines and poses an additional burden on hospital budgets.[45] Link Health Care illustrated this with a case study of the drug Defibrotide, used in the treatment of a serious complication resulting from haematopoietic stem-cell transplantation, hepatic veno-occlusive disease:

> Defibrotide is currently only available to patients under the [Special Access Scheme] and is prescribed throughout the transplantation centres across Australia. Funding is provided through the hospital budgets and a three week course of treatment can cost around $40,000 to $100,000 per patient based on the recommended daily dose of 25mg per kg.
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> The South Australia Medicines Evaluation Panel, at their meeting on 14 November 2012, recommended rejecting funding of defibrotide "due to the

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40 *Submission 142a*, Deloitte Access Economics, *Access to cancer medicines in Australia*, July 2013, p. 48;

41 *Submission 142a*, Deloitte Access Economics, *Access to cancer medicines in Australia*, July 2013, p. 47; see also: Link Healthcare, *Submission 125*.

42 Deloitte Access Economics, p. 47.

43 *Submission 152*, p. 10.

44 *Submission 146*, p. 3.

45 *Submission 125*, pp 5-6.

> number of Individual Patient Use (IPU) requests for this medicine exceeding the threshold for review as directed under SA Health policy".[46]

4.40 ANZCHOG also told the committee that it is not realistic to expect new and innovative cancer medicines to be funded from hospital operational budgets and that some hospitals have had needed to fund the supply of such drugs from donated funds.[47]

Clinical trials

4.41 Another important potential avenue for early access to new cancer medicines that have not yet received TGA approval or PBS listing is through participation in clinical trials. Clinical trials may be sponsored by pharmaceutical companies or may be initiated by researchers and health professionals.

4.42 However, as noted in chapter 3, the committee heard that there are challenges associated with clinical trials and for a number of reasons participation in clinical trials is not an option for many cancer patients:

- not all new cancer drugs are tested through clinical trials conducted in Australia;
- patients must be referred to hospitals and clinicians participating in the trial, meaning participation may not be possible due to the patient's location; and
- strict eligibility criteria may mean some patients are ineligible to participate.[48]

4.43 OCA told the committee:

> Most ovarian cancer trials are large international studies and getting a spot on one of these trials is itself fraught with difficulty. Australia is often given only a handful of spots at a few hospitals. In addition, the eligibility criteria are often so restrictive as to rule them out as an option for most women, if geography has not done that already.[49]

4.44 Mr Andrew Warden, who has been diagnosed with Waldenstrom's Macroglobulinemia (WM), a type of non-Hodgkins Lymphoma, told the committee that he had been unsuccessful in gaining access to a trial as he did not meet the eligibility criteria:

> The consensus of leading world experts identifies WM treatments including IMBRUVICA, Idelalisib, Ofatumumab, Velcade and RIBOMUSTIN. I do not have access to these treatments. There are Australian clinical trials (with limited patient intakes) for all these treatments except Ofatumumab which is only for Chronic Lymphocytic Leukaemia (CLL). My Haematologist late last year unsuccessfully sought my participation in the IMBRUVICA clinical trial. I did not then meet the specified criteria as my relapse had not

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46 *Submission 125*, p. 4.

47 *Submission 152*, p. 10.

48 CCA/COSA, *Submission 145*, p. 5; Melanoma Patients Australia, *Submission 116*, p. 3.

49 *Submission 137*, p. 4.

> then reached the stipulated level. The trial is now closing before my condition is within the defined criteria, so my chance has passed.[50]

4.45 A number of submitters called for greater access to clinical trials for Australian cancer patients.[51] Mr Anthony Steele told the committee that there is a need to facilitate increased access to clinical trials by simplifying the process for establishing trials, establishing a national ethics approval system and increasing access to international trials.[52]

4.46 Cancer Voices South Australia told the committee that clinical trials are often undertaken only in limited locations and that access often depends on patients and families searching out the information and asking clinicians to refer them to those locations.[53]

4.47 The Australian Leukaemia and Lymphoma Group (ALLG) told the committee that one of the reasons more Australian's are not able to access new drugs via the clinical trial framework is because the framework is 'slow, unresponsive to emerging trends, and focused to aid the clinical trial activity generated from the pharmaceutical industry whereby commercial outcomes of the drug in use is at the forefront of the development investment'. ALLG argued for better support for the Australian clinical trial environment, noting that:

> Cooperative clinical trial groups, like the ALLG, provide the fertile ground for patients to have access to drugs that are designed for use with the patient and their health outcomes as the focus – not the commerciality of the drug.

4.48 ALLG further stated:

> If this continues in this way, Australians will only ever continue to get access to new drugs at the discretion of a company that has valued and determined access by a commercial gain.[54]

4.49 Professor Brendon Coventry, Research Director with the Australian Melanoma Research Foundation, also noted the need for greater support in Australia for clinical trials of innovative approaches to cancer treatment. Professor Coventry, and Mr Martin Ashdown, a Research Fellow in the Faculty of Medicine at the University of Melbourne, told the committee of their research into the operation of the immune system and the importance of accurate timing of cancer treatments. Professor Coventry told the committee:

> Martin and I have subsequently identified how the immune system seems to be working, by switching on and switching off repetitively, and that when treatment occurs is vitally important. The delivery of relatively inexpensive

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50 Mr Andrew Warden, *Submission 7*, p. 3.

51 See for example: Cancer Voices SA, *Submission 150*, p. 3;

52 *Committee Hansard*, p. 11.

53 *Submission 150*, p. 2.

54 *Submission 159*.

> agents at the correct time for the patient can have a dramatic effect on the effectiveness of many therapies for cancers of many different types.

4.50 Mr Ashdown further commented:

> Continuing on, this relatively simple approach potentially offers near-immediate and less expensive opportunity to use cheaper off-patent existing drugs and some of the newer drugs more effectively. Timing of therapy seems to govern efficacy and this principle is finding its way into clinical literature year by year. Regarding the use of accurate timing of therapy, according to two highly experienced and prominent New York oncologists who have been reviewing our work, this finding has the potential to dramatically change the treatment landscape of cancer immunotherapy.[55]

4.51 The committee notes that this work has been self-funded and reliant on philanthropic support. Professor Coventry told the committee that the current grants system 'does not really serve original research and innovative research'.[56]

4.52 The committee heard that a lack of information about clinical trials may also contribute to inequities in access to clinical trials. For example, the committee heard that patients may experience difficulty accessing information about clinical trials for medicines that may be suitable for them if the trials are not offered at the hospital or oncology unit where they are receiving treatment. BCNA told the committee:

> We know that some women receiving treatment in rural and regional areas, older women and women from lower socio-economic and culturally and linguistically diverse backgrounds are poorly represented in clinical trials.[57]

4.53 The committee notes the establishment of the Australian Clinical Trials website is intended to assist patients to be aware of trials available in Australia and access information about them. The website is also intended to assist trials to recruit participants.[58]

Private funding, fundraising and charitable funds

4.54 For those cancer patients who have not been able to access treatment through trials or compassionate access, the alternatives are limited.[59] The committee heard evidence of patients travelling overseas to receive treatment at significant expense and personal cost. Submitters noted that the expenses associated with seeking treatment

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55 *Committee Hansard*, p. 10.

56 *Committee Hansard*, p. 17.

57 *Submission 90*, p. 7.

58 Joint Media Release, The Hon Sussan Ley MP, Minister for Health and The Hon Ian Macfarlane, Minister for Industry and Science, *Australian-first website to connect more patients with clinical trials*, 20 May 2015. See www.australianclinicaltrials.gov.au (accessed 15 September 2015)

59 Melanoma Patients Australia, *Submission 116*, p. 2.

overseas are 'beyond the capabilities of most Australian families' and noted the associated emotional and social burden.[60]

4.55 While the Australian Government funds a Medical Treatment Overseas Program for proven therapies not available in Australia, the committee heard that treatments on a clinical trial are specifically excluded 'presumably because by definition, if a therapy is still undergoing a clinical trial, its efficacy is not yet proven'.[61]

4.56 The committee heard that many cancer patients rely on the generosity and fundraising efforts of family, friends, colleagues and the community to finance the cost of their cancer treatment.[62] While submitters expressed their gratitude for this support, they noted the personal impact fundraising has on cancer patients and their families.[63] Mrs Naomi Brugger told the committee:

> We are fundraising at the moment. We have managed to raise close to $60,000 since January. That has been hard going. I have run the campaign mostly by myself, so that means I have been away from the boys as well. So they are not only missing out on dad; they are also missing out on mum, who is trying to keep dad alive.[64]

4.57 RCA told the committee that it has established a charitable Cancer Medicines Fund to address what it sees as profound inequities in the current system.[65] In its submission, RCA provided the committee with two examples of patient experience to illustrate how these inequities can arise:

> The first of these examples, Anita, has been diagnosed with non-small cell lung cancer and has been diagnosed as having an ALK+ genetic mutation as a contributing factor. Her oncologist prescribed a drug called Crizotinib and Anita has responded well for a number of months. Crizotinib has been recommended for listing by the PBAC for Anita's cancer but as the contractual process unfolds, it may yet take some months for it to be listed. In the meantime our Cancer Medicines Fund continues to fund her treatment at $7,400 per month.
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> Our second patient is Lillian who has also been diagnosed with non-small cell lung cancer but in Lillian's case her much rarer mutation is in the ROS1 gene. Her highly respected oncologist has also prescribed Crizotinib as there is substantial evidence of benefit. Lillian is also responding well but

60 *Submission 152*, p. 11.

61 ANZHCHOG, *Submission 152*, p. 13.

62 See for example personal accounts provided in the following submissions: Mr Jason Noble, *Submission 19*, Rare Cancers Australia, *Submission 92*, p. 19 and p. 23; Mr Matthew Story, *Submission 201*, p. 2.

63 See for example: Ms Maureen Austin, *Submission 98*, p. [1].

64 *Committee Hansard*, p. 10.

65 *Submission 92*, p. 1.

> because her cancer or indication is so rare there is currently no application to PBS for re-imbursement.
>
> Hence we face a situation where both Lillian and Anita need to self fund today at a cost of over $7,400 per month yet simply because of the random genetic mutations they have, Lillian will never receive funded medicines through the PBS whilst Anita hopefully will.
>
> Same cancer, same treatment but no fairness.[66]

4.58 The fund, which has been established under the campaign banner, is supported by fundraising, corporate support, public donation and events and campaigns under the 'Sick or Treat' banner.[67] RCA told the committee:

> That we needed to establish this site says everything we need to say about the current state of cancer medicines in Australia.[68]

Impact on rural patients

4.59 The committee heard that cancer patients living in rural and remote areas frequently suffer greater challenges in accessing cancer treatment. MA told the committee:

> There is commonly a lack of easily accessible diagnostic and treatment services in rural areas where treatment services are often rudimentary comparted to large urban centres. They will also likely lack access to current research and clinical trials, which are commonly conducted in larger urban centres. These factors contribute to later diagnosis; diagnoses at more advance stages of disease; and higher mortality rates.[69]

4.60 This is of particular concern for patients with rare or less common cancers, as rural centres are not equipped to treat less common cancers. The Unicorn Foundation, which assists and supports patients with neuroendocrine cancer, submitted that 40 per cent of neuroendocrine cancer patients live in rural areas.[70]

4.61 Medical Oncology Group of Australia (MOGA) and LFA expressed concern that cancer patients receiving treatment outside major treatment centres, such as those from regional and rural areas, may also experience difficulty accessing compassionate and early access programs and clinical trials.[71] LFA told the committee :

> In our survey, of the patients who accessed a new drug, 17% had to relocate for treatment at their own cost. However, none of the State or Territory Patient Assisted Travel Schemes (PATS) provides a subsidy to cover rural, regional or remote patients to travel to a metropolitan hospital to take part

66 *Submission 92*, p. 9.

67 Cancer Medicines Fund, www.sickortreat.org.au (accessed 7 September 2015)

68 *Submission 92*, p. 1.

69 *Submission 142*, p. 25.

70 *Submission 130*, p. 2.

71 *Submission 108*, p. 2; *Submission 123*, p. 12.

> in a clinical trial. Even if the clinical trial covers the cost of the drug, rural, regional and remote patients must bear ongoing accommodation and travel expenses as well as the cost of other medications. It means accessing new drugs through clinical trials is an inequitable, user-pays system that penalises non-metro patients and their families.[72]

4.62 LFA underscored this evidence with the following four patient case studies, which demonstrate the additional challenges faced by rural and regional cancer patients seeking to take part in clinical trials:

> **Patient case study 6:**
>
> I made the decision to move from Canberra to Melbourne, where I had no family, to avoid having to commute regularly to receive the drug (which had to be given intravenously as part of the clinical trial program). This was a very hard thing to do, but looking back it doesn't seem there really was any other option for me at the time.
>
> **Patient case study 7:**
>
> My wife and I had to live in Melbourne for 14 weeks while undergoing the treatment, which wasn't available in our regional centre, 300 km from Melbourne. Our first month cost $5550 for accommodation. Thereafter we were lucky enough to gain access to Leukaemia Foundation accommodation, which meant a saving in the order of $10,000.
>
> **Patient case study 8:**
>
> My treatment regime involved two consecutive days each four weeks. It was too far (and way too exhausting) to travel to and from home two days in a row so I stayed in a motel located near the hospital. It was pretty lonely going back to a motel after an exhausting day of treatment, monitoring any symptoms and then going back in a taxi the next day to do it all again.
>
> **Patient case study 9:**
>
> I'm taking part in an international clinical trial. This requires driving for treatment every two weeks to Gosford from Tamworth. While in Gosford I live in in rented accommodation. Fortunately, our children are old enough to be self-sufficient and my wife's still working, so we're able to pay for the costs from her personal savings. So far, the treatment's been successful.[73]

4.63 The committee notes that for families of young cancer patients the only option may be to relocate the whole family to be nearer to treatment and support:

> To undergo this intense treatment schedule, Zoe's family had to leave their farm – 18,000 acres of mixed cropping and livestock – and relocate to Perth to be closer to the Princess Margaret Hospital for Children. Zoe's older sister…and little brother…(who was born just a few months after Zoe's

72 *Submission 123*, p. 12. The Leukaemia Foundation undertook a survey on new and innovative drugs of 675 blood cancer patients (or their carer) in February 2015, *Submission 123*, p. 2.

73 *Submission 123*, p. 12.

> diagnosis), together with her mum…and dad...fought with Zoe all the way.[74]

4.64 Roche Products submitted that the potential benefits for regional and rural patients of new forms of cancer therapy should be accorded greater value in the assessment process. For example, Roche Products stated that 'oral and sub-cutaneous forms of intravenous cancer therapies may be more easily used outside of major metropolitan hospitals and may support patients completing their full course of therapy'.[75]

4.65 Mrs Jill Delahoy's experience taking the drug Ibrutinib as part of her treatment for Chronic Lymphocytic Leukaemia also highlights the impact some new cancer medicines can have on both the quality of care and quality of life for rural patients:

> This drug comes in capsule form and is taken by the patient at home. This means no hospital admission required, either for day chemotherapy or by admission. My haematologist is of the view that in a short time this will become the normal treatment for CLL patients. Imagine what this means for rural residents, no going to hospital which may be many hours away at a time when the patient is feeling dreadful. This should reduce costs and pressure on the hospital system.[76]

Impact on patients with rare cancers

4.66 The committee heard that, as research priorities, commercial imperatives and advocacy tend to focus on oncology medicines and treatments for more common cancers, the impacts of a lack of access to subsidised cancer medicines has a disproportionate impact on the quality of care for patients with rare and less common cancers, for whom there are often few treatment options.[77]

4.67 The evidence base for rare cancers, which have small patient populations, is more likely to have some level of uncertainty.[78] Clinical trials for rare cancers are often conducted through collaborative trial groups with less industry support and the data collected may be less suited to registration and reimbursement requirements.[79]

4.68 Dr Christopher Fraser, Chair of ANZCHOG, told the committee that most drugs for rare cancers, which includes most childhood cancers, are not covered by the PBS and are not likely to be:

> The problem is, to get to that PBS approval, the data is almost never going to be available to prove clinical and cost-effectiveness in extremely small patient groups, and the economic incentive to try and collect the data is not

74 The Kids Cancer Project, *Submission 192*, p. 9.

75 *Submission 114*, p. 11.

76 *Submission 79*, p. 1.

77 MOGA *Submission 108*, p. 3.

78 Cancer Action Victoria, *Submission 151*, p. 5.

79 MOGA *Submission 108*, p. 3.

> going to be available. The drug company is going to say: 'Let's apply for a listing for breast cancer.' They are not going to be worried about a rare liver tumour in children. At the moment in our system we have to beg our hospital executives to pay for these drugs, and they may or may not approve it. There are inequities amongst different state thresholds. For rare diseases, like just about every childhood cancer, we feel there needs to be an improved mechanism that assesses the eligibility for federal funding or a federal subsidy for these drugs that does not rely upon extremely stringent clinical and cost-effectiveness data and that is not subject to the vagaries of individual jurisdiction or approval.[80]

4.69 In its submission, the Department of Health noted the difficulties associated with funding for treatments for rare cancers:

> Making judgements about the level of support for rare cancer patients is especially difficult, noting that it involves spending significant amounts of taxpayer dollars on a very small, but very sick, sub-group. The trend in increasingly expensive, personalised medicines will continue to place pressure on both the family and national budgets.[81]

4.70 As Ms Lee McKerracher submitted, an individual's access to medicines should not be dictated by the type of cancer they have or their financial position:

> For those diagnosed with a rare cancer, they are in a different position. They are not lucky like I was and have ready access to a range of therapies that are reimbursed. So in addition to the emotional stress they are under, these people need to try and fund treatments to get well. These treatments can be extremely expensive and many can't afford them. Access to medicines should not be dictated by the type of cancer someone has, nor the income they earn. These people do not put their hands up to get cancer, they are a victim of circumstance and should not be discriminated against just because their disease is rare.[82]

4.71 A number of submitters argued that alternative approval and funding pathways are required to address the particular challenges faced by patients with rare cancers. For example, LFA proposed that consideration be given to automatic conditional acceptance of treatments for rare cancers that have been approved in the United States and Europe. Mr Steele told the committee:

> We know there are some life-saving drugs that may be available to rare cancers but are not brought into the country because it is not profitable. We would like to have some other methods to access those drugs for the non-profitable therapies to the rare cancer groups.[83]

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80 Dr Christopher Fraser, Chair, Australian and New Zealand Children's Haematology and Oncology Group, *Committee Hansard*, pp 35 -36.

81 Department of Health, *Submission 197*, p. 20.

82 *Submission 25*, p. 1.

83 *Committee Hansard*, p. 12.

4.72 The account of Mr John Canning summarises the sentiments of many submitters. Mr Canning has inoperable metastatic stage 4 lung cancer. Despite his prognosis, Mr Canning describes himself as mentally and physically fit. Mr Canning has a rare genetic mutation known as anaplastic lymphoma kinase and is being treated with the drug crizotinib:

> What does this mean for me? I actually do not feel like a patient. I can take this drug orally, in the form of a tablet twice a day wherever I am, whether I am travelling or out at dinner with family and friends. If I was on the current PBS listed alternative pemetrexed, I would have to book in for an infusion at a public hospital or cancer centre every two to three weeks. The side effects of the targeted therapy that I am on have been proven and shown to be much milder and much more manageable. I have some control over my life as a cancer sufferer. I have quality of life. For me and for patients like me, that is what it is all about: quality of life.
>
> There is no plan B. At this point in time, I do not have other options. I am still working, albeit fewer hours. I continue to carry out the charitable and pro bono work that I have done during my professional career. It means an enormous amount to me emotionally, psychologically and physically, but it makes an enormous difference to my wife and my family, who are my primary carers.
>
> There is a challenge with all this because, although crizotinib was approved at PBAC's November meeting, it is still not listed on the PBS. It is not reimbursed. It is an expensive drug. I am one of very few people in this community who can afford to purchase this drug. For every one of me, there are 30-plus people who cannot. There are currently around 340 people with my cancer in Australia today. There are only around 40 who are likely to be diagnosed each year. What we are after is a fair access scheme for people with rare cancers. It is not an expensive access scheme but it is one which means a truly remarkably different experience than the brutal, debilitating and exhausting process of normal cancer chemotherapy treatments.[84]

4.73 Evidence to the committee indicated a move toward more personalised use of existing medicines in the treatment of rare cancers. For example, CBCF told the committee that it is tackling the paucity of medicines to treat brain cancer through:

- A significant, increasingly global, research collaboration, to accelerate new treatment options for patients.
- A concerted move towards a 'personalised medicine' approach, whereby tumour genetics are established early on and high-throughput screening of existing medicines (many of which are currently PBS listed for other indications) occurs. If any of the screened drugs show activity against an individuals' tumour, then this information is conveyed to the treating

84 *Committee Hansard*, p. 41.

> oncologist (and hopefully patient) to be used for (most likely off-label treatment.[85]

4.74 As noted in chapter 2, commercial realities often mean that pharmaceutical companies do not seek registration of cancer medicines for rare indications with small patient populations. Rare Cancer Australia (RCA) told the committee:

> The potential outcome is that today we have medicines registered with the TGA for one indication meaning they are deemed safe and for that indication also efficacious. However, their utilisation for other indications is prevented simply by the lack of application for registration.[86]

4.75 RCA expressed the strong view that where a medicine is already registered in Australia for one indication, clinicians/and or patient groups ought to be able to apply to the TGA to extend the registration to additional indications where reasonable evidence of efficacy exists.[87]

Impact on children, adolescents and young adults

4.76 The committee notes that the concerns raised above are amplified in the case of young cancer patients. The committee also received evidence that children, adolescents and young adults (AYAs) diagnosed with cancer face unique challenges in accessing new, innovative and specialist cancer drugs.

4.77 The committee heard that childhood cancers are different from adult cancers. While the most common form of adult cancer is carcinoma, this form of cancer constitutes less than three per cent of paediatric cancers. ANZCHOG told the committee:

> Consequently, the molecular targets seen in childhood cancers are often different to those in adult cancers. However, currently the approach taken to applying targeted therapy to paediatric oncology is to see which drugs being developed for adult cancers might have some activity in children. The paediatric oncology community would argue that children deserve their own drugs developed specifically for their diseases. The explosion of new agents, a relative lack of pre-clinical research and the small numbers of paediatric patients makes prioritisation of agents for clinical trials extremely challenging.[88]

4.78 CanTeen submitted that AYAs face exceptionally difficult cancer journeys, noting that the number of young people aged between 15-24 years is higher than in the younger age groups. AYAs present with a greater range of cancer types and, due to delays in diagnosis, the prognosis for AYAs is not as good:

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85 *Submission 187*, p. 1.

86 *Additional Information*, received 16 September 2015.

87 *Additional Information*, received 16 September 2015.

88 The Australian New Zealand Children's Haematology/Oncology Group in partnership with The Kids Cancer Project, The Kids Cancer Alliance and the Children's Cancer Institute (ANZCHOG) *Submission 152*, p. 1

> The number of young people aged 15-24 years diagnosed with cancer is 1.5 times the number of children aged 0-14 years that are diagnosed. Young people have significantly poorer survival rates than children and older adults in some of the cancers common in this age group. Many of the cancers that affect young people are rare. Young people also present with a larger array of cancer types compared to older adults: 90% of the cancer burden is accounted for by 20 different cancer types. Furthermore, young people also tend to present with cancer at a more advanced stage due to longer delays before diagnosis and suffer higher rates of inferior psychological outcomes compared to other age groups. This in turn, is associated with poorer prognosis and a heightened risk to survival. Consequently, for some cancers, young people show a much poorer response with the same treatments given to older adults or younger children.[89]

4.79 Similarly, AYAs encounter disproportionate difficulty in accessing new and innovative cancer medicines. There is a lack of clinical trials for the cancer subtypes commonly seen in AYAs because pharmaceutical companies do not usually devote the same research effort to rare diseases. CanTeen noted that, unlike the United States and Europe, where legislation has been passed encouraging pharmaceutical companies to develop drugs with paediatric indications, Australia has not provided a legislative incentive for pharmaceutical companies to seek the PBS listing of indications relating to cancers in children and adolescents.[90]

4.80 One young submitter told the committee:

> There are no drugs for kids like Eva to try and save their life. The big drug companies need help from the government to find better ways to help kids get new drugs. The government needs to help out scientists find drugs for little kids so they don't just have to use drugs for old people. Kids are different and the government needs to treat them differently to grown up people.[91]

4.81 The Kids Cancer Project submitted:

> Australia now needs to develop a policy for incentives and/or require industry to participate in the supply of drugs for childhood cancer and eliminate inequities. The fear is that Australia will not keep pace internationally with improvements in survival rates and children and families will continue to seek international options.[92]

4.82 The rarity of the cancers that affect children and AYAs means it is extremely challenging to conduct trials necessary to satisfy the requirements to achieve

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89 *Submission 146*, p. 2.

90 See for example: Best Pharmaceuticals for Children Act, 2002, Paediatric Research Equity Act, 2003, and European Paediatric Medicine Regulation, 2007.

91 Name withheld, *Submission 115*, p. 2. The submitter is 12 years old. Her six year old sister Eva died from rhabdomyosarcoma in 2014.

92 *Submission 192*, p. 6.

subsidised funding through the PBS.[93] It is not economically attractive for pharmaceutical companies to invest in drug development for childhood cancers:

> The success of frontline therapy and the fact that new therapies target smaller sub-groups of already rare diseases means that there are fewer patients eligible to test these therapies and makes the conduct of trials to prove clinical effectiveness extremely challenging.[94]

4.83 While noting the dramatic improvement in survival rates for children with cancer 'from less than 30% in the 1960s to 80% currently', ANZCHOG states that the most important factor in this improvement, has not been access to new cancer medicines, but global collaboration in incorporating clinical research as an essential part of the care of children.[95] The committee heard that all of Australia's major paediatric hospitals have access to international trials and it is estimated that more than 50 per cent of Australian children with cancer will participate in a US or European originated children's cancer clinical trial.[96]

4.84 However, meeting the costs associated with treatment of childhood cancers poses significant challenges for hospital operation budgets. ANZCHOG told the committee 'most Australian paediatric oncologists would be very reluctant to ask a family to pay for a high cost therapy themselves because of the ethical challenges that this presents'.[97]

4.85 Reliance on childhood cancer clinical trials based and funded in the United States and Europe is also not without its complications. Unlike adult trials, which are often initiated by the pharmaceutical industry, childhood cancer clinical trials are initiated by organisations such as the United States based Children's Oncology Group and the European based Consortium for Innovative Therapies for Children with Cancer funded by the United States and European governments. The committee heard that Australia's access to some childhood cancer clinical trials funded by the Children's Oncology Group is being limited due to budgetary constraints.[98]

4.86 The committee also heard that there can be difficulties associated with children under the age of 18 gaining access to trials. CBCF noted that children are often not allowed to enter trials because the medicines must be tested on adults first.[99] CanTeen argued that there is no valid biological justification for age-eligibility criteria and told the committee that the practice has been criticised internationally by AYA

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93 *Submission 152*, p. 1.

94 ANZCHOG, *Submission 152*, p. 1.

95 *Submission 152*, p. 1

96 Kids Cancer Project, *Submission*, 192, p. 15.

97 *Submission 152*, p. 10.

98 Kids Cancer Project, *Submission* 192, p. 5; CanTeen, *Submission 146*, p. 3.

99 Ms Michelle Stewart, Head of Research Strategy, Cure Brain Cancer Foundation, *Committee Hansard*, p. 14.

cancer advocacy groups.[100] The Kids Cancer Project told the committee that decisions to register and subsidise drugs for use in adults but not children can also lead to inequities. For example, the drug clofarabine, used in the treatment of acute lymphoblastic leukaemia, is subsidised for use in adults but not in children. The annual cost of treatment for a child can be as much as $100 000 compared to $37 for an adult.[101]

4.87 Finally, as noted earlier, many childhood cancers are rare and patient populations are small. This lack of a commercial incentive can hamper the development of trials to test the effectiveness of drugs used in the treatment of adult cancers in treating the types of cancers seen in AYAs. For example, CanTeen said Ruxolitinib, a drug developed for older adults with myeloproliferative disease has been reported to be effective in some AYAs with Philadelphia-like acute lymphoblastic leukaemia. Despite the apparent effectiveness of this drug in treating this disease, CanTeen stated it is unlikely that the PBAC will approve the indication without clinical trials and there is currently no incentive for pharmaceutical companies to undertake trials in such a comparatively small market.[102]

Committee view

4.88 The committee notes that for cancer patients and their families, maintaining a normal life and enhancing the quality of that life is of utmost importance. The uncertainty and significant financial cost associated with off-label use of cancer drugs results in significant physical, emotional and financial stress associated with the uncertainty of securing that access. As Ms Robyn Lindley noted:

> You can't work, your husband needs to work less to help so his income is lower. Then you also have the expenses of pharmacy products to counteract side effects. So where do we get this money from? You're so worried about surviving and beating this dreaded disease and wondering if you are going to be here for your children to then add another huge stress of MONEY.[103]

4.89 The committee understands that often the patients with the highest medical need are also the patients with the least capacity to fund their own treatment.[104] Many patients are already experiencing financial difficulty and do not have the capacity to meet significant out of pocket costs. Access to new treatments through clinical trials and compassionate programs is by no means certain, and in the case of the latter, can involve a significant contribution on the part of the cancer patient. The burden is even greater for those cancer patients with rare or less common cancers, particularly children and young adults, or who live in rural and regional Australia, who have fewer treatment options to begin with.

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100 *Submission 146,* p. 3.

101 *Submission 192*, p. 6.

102 *Submission 146,* p. 2.

103 Quoted in Bowel Cancer Australia, *Submission 149*, p. 4.

104 Mr Paul Hobson, *Submission 6*, p. 2.

4.90 Special access schemes, compassionate access programs and clinical trials are an important source of assistance, but help only a small minority of cancer patients and are neither sustainable nor equitable alternatives to subsidised access through the PBS. Over reliance on such schemes also risk creating a two tiered health system in which some cancer patients can afford treatment and others cannot.[105]

4.91 The committee is particularly concerned about the impact of delays in access to innovative cancer medicines to treat patients diagnosed with rare and less common cancers, including children and young people and those with brain cancers. Proportionally, research in these areas of cancer research is less well supported than other areas of cancer research, while at the same time expenditure on treatment for these forms of cancers is much greater. For example, the committee notes that treatment of brain cancer costs more per person than any other form of cancer, yet receives only a small fraction of Australian Government cancer research funding.[106]

4.92 In this context, the committee recognises the importance of supporting innovative research that may lead to more effective use of existing cancer treatments with consequential savings in the longer term. The committee notes that work described by Professor Coventry and Mr Ashdown in relation to a locally produced vaccine to treat advanced metastatic melanoma highlights the potential for the delivery of relatively inexpensive agents at the correct time for an individual patient to have a dramatic impact on the effectiveness of many therapies for many different types of cancers.[107]

4.93 The committee considers the long-term implications of research of this type merit further investigation. Similarly, the committee considers that there is merit in considering the extent to which legislative and other incentives implemented overseas might have application in an Australian context to encourage greater focus on the development of cancer treatments for rare and less common cancers.

105 Takeda Pharmaceuticals Australia Pty Ltd, *Submission 122*, p. 4.

106 Cure Brain Cancer Foundation, *Submission 187*.

107 *Committee Hansard*, p. 10.