Chapter 6
Conclusions and recommendations

6.1 As noted in this report, Australia has the highest incidence of cancer in the world. While Australia also has some of the best cancer survival outcomes in the world, the provision of timely and affordable access to new and innovative cancer medicines provides a significant challenge for the Australian Government, clinicians and patients. These challenges stem in part from the fact that cancer medicines are among the most expensive medicines, and from Australia's relatively small patient populations.

6.2 These challenges are also a consequence of an increasingly sophisticated understanding of cancer as not one, but many hundreds of diseases requiring an equally sophisticated and individualised method of treatment. The committee heard that advances in the treatment of cancers are frequently incremental and increasingly targeted at small patient populations. More targeted medicines and therapies have the ability to increase the range of treatment options for cancer patients, resulting in improved quality of life and survival for many patients. At the same time, cancer is an area of high clinical need meaning that, even with access to subsidised medicines, many cancer patients face significant financial hardship. These challenges are exacerbated for those patients with rare or less common cancers, particularly children and young people, and those who live in rural and remote communities.

6.3 These factors pose a significant challenge for all governments as they seek to facilitate affordable cancer care while maintaining the sustainability of the overall health budget. The current trends in cancer research can be expected to continue.

6.4 Throughout this inquiry, the committee has been acutely aware that cancer patients are not the only patients who experience difficulty in accessing new and innovative medicines in a timely way. The committee considers that the concerns identified in this inquiry could easily apply to those diagnosed with a range of chronic or less common diseases. What sets cancer patients apart from many other patients is time. The vast majority of cancer patients do not have time on their side.

6.5 The committee considers that if the process for the assessment and listing of medicines can be enhanced to address the particular concerns that arise in relation to cancer medicines, it will inevitably serve the needs of all Australians more effectively.

6.6 Evidence to the inquiry has underscored the fact that access to medicines ultimately depends on the ability of patients to pay for them. The listing of medicines on the Pharmaceutical Benefits Scheme (PBS) plays a significant role in ensuring this access is equitable for all Australian patients.

6.7 As mentioned earlier, evidence to the inquiry has demonstrated that for many cancer patients access to new and innovative treatments comes at significant personal and financial cost. Those who require access to cancer medicines not currently listed on the PBS must resort to access through compassionate programs or clinical trials.

Evidence to the committee has demonstrated these avenues of access are neither equitable nor certain and frequently incur significant cost.

6.8 The committee heard that the inability to access cancer medicines, either because the preferred course of treatment is not registered in Australia or is not subsidised via the PBS, has significant flow on consequences for cancer patients and the people who care for them. The committee received numerous accounts describing the personal experience of cancer patients. These accounts underscored the grim reality that for cancer patients delays in access to new and innovative cancer medicines can be measured in loss of quality life years and lives lost.

6.9 The committee notes that a key factor in the timely availability of new cancer medicines is the timing of applications for registration and reimbursement by pharmaceutical companies. This is a commercial decision made in the context of a global industry. The committee understands the commercial imperatives that may lead a pharmaceutical company to seek regulatory approval in the United States or Europe in the first instance, in preference to a country with a small population, like Australia.

6.10 However, while the timing of the lodgement of applications is outside the control of Australian regulatory authorities, the committee notes that there is scope for the Australian Government to ensure that the regulatory processes in place for the consideration of applications are efficient and do not act as a disincentive to companies to seek listing and reimbursement.

Enhancing the operation of the TGA and the PBAC

6.11 Evidence to the inquiry has stressed the value that stakeholders place on the PBS and the Pharmaceutical Benefits Advisory Committee (PBAC) system. Submitters noted the importance of decisions regarding the registration and reimbursement being based on a rigorous, evidence based assessment of safety, efficacy and value.

6.12 However, while submitters consistently emphasised that the current PBS and PBAC process has served Australia well, they also emphasised the need for the system to be reviewed to ensure that it is capable of dealing with the challenges posed by the rapid development of cancer treatments in particular.

6.13 While some submitters expressed concern that a one-size-fits-all assessment process is no longer fit for purpose, the committee considers that the concerns raised in relation to the current process should be able to be addressed without creating a parallel process. The committee also notes that there is considerable commitment and goodwill within the pharmaceutical industry and the stakeholder community to work with government to explore avenues for addressing these concerns.

More streamlined and flexible processes

6.14 While the committee has noted that the current assessment processes are appropriately rigorous and based on clearly cyclical timelines, the committee notes that there is scope to improve the timelines for consideration of applications. The committee also notes that greater flexibility regarding evidentiary requirements and provision for entities other than the sponsor of a medicine to seek registration of new indications for medicines in appropriate circumstances may address concerns

regarding the responsiveness of the current registration system to changes in the clinical setting.

6.15 The committee notes evidence to the inquiry regarding fast track processes employed by overseas regulators and has noted that key features of such programs are early and frequent engagement between the regulator and the sponsor to address any issues associated with assembling data in support of an application and some form of 'rolling review'.

6.16 The committee also notes concerns regarding the evidential requirements of the current system. The committee considers that greater formal emphasis should be placed on quality of life considerations. In this context, the committee welcomes the current review of Parts II and III of the PBAC Guidelines and notes that the review provides a timely opportunity clarify the information requirements for applications for PBAC assessment. The review also offers an opportunity to identify new developments with regard to current methodology, along with any issues of scientific debate and consideration of Australian and international best practice.

6.17 The committee also supports greater collaboration between the TGA and the PBAC, along with continued examination of current parallel processing arrangements, to identify options for streamlining processes and minimising duplication in order to achieve compressed timeframes where possible.

Improved managed access programs

6.18 The committee notes the potential for managed access programs to address some of the concerns raised in relation to evidential requirements while at the same time providing more timely access to subsidised medicines. While Australia's initial managed entry scheme has not been enthusiastically embraced, the committee welcomes the work of the Access to Medicines Working Group (AMWG) in developing a new framework for a managed access program. The committee encourages the AMWG to consult closely with clinicians and consumers in finalising the framework.

6.19 The committee also notes evidence emphasising the need for consideration of a number of possible avenues to address demand for early access to new medicines. The committee notes that the provision of sustainable subsidised access to medicines, particularly expensive cancer medicines, will continue to pose a significant challenge for the Australian Government. The committee therefore supports the examination of a range of possible access models.

An increased role for consumers and clinicians

6.20 The committee considers that consumers and clinicians should play a more substantial role in the evaluation of new medicines. The committee commends the PBAC for its efforts to facilitate consumer engagement through the introduction of consumer and patient hearings.

6.21 The committee considers that consideration should be given to avenues for facilitating more formal discussion with the Australian community. The committee notes evidence received regarding the operation of formal mechanisms overseas to capture community expectations around broader moral and ethical considerations and

considers there is merit in considering how similar mechanisms might operate in the Australian context.

Greater transparency

6.22 The committee considers that greater transparency throughout the regulatory system will enhance the engagement of all stakeholders and will support a clearer understanding of the reasons for delays in listing of particular cancer medicines. Greater transparency also has the potential to support greater procedural efficiency and a commitment continuous improvement.

6.23 The committee notes the PBAC's commitment to increasing the transparency of its processes and the level and clarity of information available to consumer and patient groups. The committee notes the implications of commercial in confidence considerations for these initiatives, but encourages the PBAC and industry to work together to address these.

Improved monitoring and data collection

6.24 The committee notes the importance of establishing effective mechanisms for collecting and analysing clinical data in relation to the use of cancer medicines.

6.25 Evidence to the committee has underscored the importance of effective review of medicines after their listing on the PBS as a means of supporting the listing of medicines through managed entry programs. The committee welcomes the new guidance for post market reviews produced by the AMWG. The committee encourages the AMWG to continue to consult widely on the operation of the post market review program as greater use is made of managed access programs and more flexible assessment criteria to explore ways in which the program could support such initiatives.

6.26 The committee notes calls for the establishment of a national cancer registry, and, while it sees merit in this proposal, considers that a review of existing data collection mechanisms is a necessary precursor to the establishment of such a registry. The committee considers that a review of data collection must consider options for linking existing databases, facilitating wider access to the data collected and avenues for collecting data regarding the off-label use of cancer drugs.

The case for an interim specialist cancer drug fund

6.27 Evidence to the committee stressed that, while a comprehensive review of the current PBAC processes was necessary, such a review would take time to complete and cancer patients do not have time on their side. Submitters advocated the introduction of an interim cancer drug fund pending completion of a review, particularly for patients diagnosed with rare cancers.

6.28 The committee is cautious around suggestions that advocate for the establishment of separate regulatory mechanisms specifically to deal with cancer drugs. The committee is mindful of concerns raised about the operation of such funds overseas. In particular the committee is concerned at the potential for such funds to exacerbate some of the issues identified with the current PBAC system around cost

and access to cancer medicines, and the impact of separate assessment processes on the rigour and integrity of the PBAC system.

6.29 The committee notes NHS England's current review of its Cancer Drug Fund and the unintended consequences arising from the operation of the fund. The committee notes that NHS England is considering a managed access pathway as an alternative to a cancer fund.

6.30 The committee considers that if such a fund were to be established, it is preferable that it is established within the current regulatory framework and operates consistently with existing processes. The committee considers that the current Life Saving Drugs Programme (LSDP) may offer a basis for the delivery of an expanded government funded compassionate access program for patients with rare or less common cancers.

6.31 The operation of the LSDP is currently the subject of a post-market review. While a technical assessment of the LSDP has raised questions regarding the sustainability of the program in its current form, it has also highlighted options for enhancing its operation. The committee considers that there is merit in drawing on this current review to examine the scope for modifying the administration of the LSDP to provide an interim means of subsidised access to medicines for the treatment of rare cancers.

The need for a coordinated review of access pathways for cancer medicines

6.32 The findings of this inquiry are not new. Similar findings have been identified in previous reviews initiated by the Parliament and the Australian Government. However, the evidence to this inquiry has underscored the importance of acting to address the concerns raised in order to ensure that Australia has a system that is capable of meeting both the challenges posed by rapid developments in medical research and the demand for subsidised access to new and innovative medicine in a way that is timely, equitable and sustainable.

6.33 The committee has acknowledged that the current work of the independent Review of Medicines and Medical Devices Regulation also overlaps with the terms of reference of this inquiry and has produced findings that are consistent with the evidence the committee has received. The committee notes that the review panel has made recommendations to:

- expand the pathways by which sponsors can seek marketing approval for a medicine or medical device, including making provision for utilisation of assessments conducted by comparable regulators, and for expedited assessments in defined circumstances;
- identify comparable overseas national regulator authorities using transparent criteria;
- enhance post-market monitoring of medicines and medical devices and streamline post-market requirements in respect of products in the Australian Register of Therapeutic Goods; and

- improve transparency and predictability of processes and decisions to build trust and confidence in the NRA's ability to ensure Australians have timely access to high quality, safe and efficacious products.[1]

6.34 The committee urges the Australian Government to give careful consideration to the implementation of these recommendations.

6.35 The committee also acknowledges work undertaken by the pharmaceutical industry and other key stakeholders. In particular, the committee notes the outcomes of the work streams initiated by the Cancer Drugs Alliance as a result of its forum in March 2014. The work of the AMWG in relation to the managed access program, transparency of PBS processes and post-market reviews, also has the potential have a positive impact on access to new cancer medicines. The committee considers that this work within the stakeholder community speaks to the considerable value placed on the PBAC system and the commitment and good will expressed by all stakeholders to working closely with government to improve its operation.

6.36 The committee has also noted initiatives that have the potential to impact on the assessment of medicines for listing on the PBS. While some of these, such as the review of the PBAC Guidelines and initiatives to enhance consumer engagement throughout the PBAC process, are positive interim steps towards enhancing the operation of the current system, the impact of others, such as the Pharmaceutical Benefits Scheme Access and Sustainability Package, are not yet known.

6.37 The committee recognises the importance of timely, interim changes but is concerned that an incremental approach to reform in this area risks being piecemeal and may squander the opportunity to identify synergies and efficiencies that a more coordinated and comprehensive review could identify. The committee considers that it is incumbent on the Australian Government to respond to the challenges facing the operation of the PBAC and the ongoing sustainability of the PBS in a comprehensive and considered manner.

6.38 The committee also wishes to emphasise the importance of consulting widely in the development and implementation of changes to the current system. In particular, while the committee welcomes the work of the AMWG, the committee encourages broader consultation with all relevant stakeholders prior to the implementation of changes as a result of the AMWG's work program.

6.39 Finally, as noted above, while this inquiry has focussed on access to cancer medicines, the committee considers that its findings have broader application. A review that seeks to address the concerns raised with regard to access to new and innovative cancer drugs, will inevitably address the concerns of all of those patients who rely on the PBS for timely and affordable access to best practice medical treatment.

Recommendation 1

1 Review of Medicines and Medical Devices Regulation, Report on the regulatory framework for medicines and medical devices, March 2015, www.health.gov.au (accessed 14 September 2015).

6.40 The committee recommends that the Australian Government initiate a comprehensive review of the system for the registration and subsidisation of medicines. The review should examine:

- **all available pathways for the registration and listing of new medicines, or new indications for medicines already registered on the ARTG and listed on the Pharmaceutical Benefits Scheme, including making provision for utilisation of assessments conducted by comparable overseas regulators; provision for clinicians and/or patient groups to apply for an extension of existing registrations to additional indications, managed access programs and risk-sharing, and the adoption of more flexible evidential requirements;**
- **options for improving the operation of assessment processes including:**
  - **enhancing engagement with sponsors and other stakeholders to better tailor their applications to the requirements of the PBAC, including consideration of pre-application planning meetings;**
  - **applying tiered assessment processes as a means of matching resources to the complexity of applications;**
  - **encouraging greater cooperation between the PBAC, the TGA and the Medical Services Advisory Committee, including examination of options for enhancing the operation of parallel processing arrangements; and**
  - **ensuring greater transparency throughout the assessment process;**
- **options for expanding the post-market review of medicines;**
- **enhancing and formalising mechanisms for consumers and clinicians to play a more central and substantial role in the evaluation of new medicines and new indications for already listed medicines, including:**
  - **consideration of options for expanding consumer and clinician representation on the PBAC;**
  - **enhancing existing avenues for stakeholder input, including the use of consumer and patient hearings; and**
  - **avenues for incorporating public perspectives on overarching moral, ethical and opportunity cost considerations into PBAC decision making processes, including consideration of models employed by comparable overseas regulators; and**
- **options for ensuring that the necessary administrative and technical resources are available to support the implementation of an enhanced PBAC system.**

Recommendation 2

6.41 The committee recommends that the Australian Government commission a review of current data collection mechanisms for cancer medicines, including identification of:

- **obstacles to the integration of existing databases and potential avenues for addressing these;**
- **opportunities to incorporate data from post-market evaluations; and**
- **avenues for capturing data relating to the off-label use of cancer medicines.**

Recommendation 3

6.42 The committee recommends that the Australian Government establish a Steering Committee to examine the feasibility of establishing a national register of cancer medicines.

Senator Rachel Siewert

Chair