

## Chapter 5

### Alternative access models for new and innovative cancer drugs

5.1 As noted in earlier chapters, while submitters consider that the current system for providing subsidised access to medicines has served Australia well, they also consider it is in urgent need of review and modernisation. Submitters noted that the particular plight of cancer patients highlights the need for a substantive overhaul of the current system to improve flexibility and timeliness of decisions, while retaining the rigour of the existing process.<sup>1</sup>

5.2 Throughout the inquiry, the committee heard a range of evidence regarding approaches used by other countries that might be considered as models for alternate approaches to providing access to cancer drugs. Submitters noted that some countries have introduced specialist mechanisms to facilitate wider and more-timely access to cancer drugs. For example, the United Kingdom established the Cancer Drugs Fund (CDF) in 2010 as a temporary fund to meet the costs of some cancer drugs either rejected by the National Institute for Health and Care Excellence (NICE) or not yet evaluated by them.<sup>2</sup>

5.3 As noted in Chapter 1, Canada established the pan-Canadian Oncology Drug Review as a cancer specific national drug review process separate from the Common Drug Review (CDR) in response to concerns that the CDR process for consideration of oral oncology products was not meeting the needs of patients and clinicians.<sup>3</sup>

#### Establishment of a specialist cancer drug fund

5.4 Many submitters emphasised the need for Australia to implement a specialist early access program for cancer drugs. However, a number of submitters recommended the establishment of such a fund as a temporary measure pending 'modernisation' of the Pharmaceutical Benefits Scheme (PBS) and Pharmaceutical Benefits Advisory Committee (PBAC) processes.<sup>4</sup>

5.5 The Private Cancer Physicians of Australia (PCPA) expressed support for the formation of a separate, novel funding mechanisms for high cost drugs outside the PBS prior to drugs being considered by the PBAC. PCPA proposed that once a drug

---

1 See for example: Rare Cancers Australia (RCA), *Submission 92*; p. 10; Pharmaceutical Society of Australia (PSA), *Submission 176*, p. 6.

2 Department of Health (DOH), *Submission 197*, p. 17.

3 Angela Rocchi, Isabelle Chabot and Judith Glennie, Evolution of health technology assessment: best practices of the pan-Canadian Oncology Drug Review, *Clinicoecon Outcomes Res.* 2015, 7:287-289, <http://www.ncbi.nlm.nih.gov/pmc/articles/PMC4461134/> (accessed 7 September 2015)

4 See for example, Mr Peter Carr, *Submission 13*, p. 1; See also Cancer Drugs Alliance, *Improving Access to Cancer Medicines*, White Paper, March 2015, p. 25.

receives PBAC approval, such funding would no longer be required and suggested that cost recovery mechanisms could be applied in the event that a drug failed to gain approval.<sup>5</sup>

5.6 The Tasmanian Government submitted that a cancer drug fund which supports access to cancer medications that are not available via the PBS, like that established by the National Health Service (NHS) in England, could provide an expedient way of enabling access to cancer medications and could be preferable to the creation of separate administrative arrangements for specific cancer medicines:

[T]he creation of new administrative arrangements for some newly listed medicines, as has been seen with the introduction of Herceptin and more recently Eculizumab, should be avoided as it created an additional level of administrative burden and access ambiguity for clinical staff and patients to overcome.<sup>6</sup>

5.7 Medicines Australia (MA) told the committee that the UK and Canada, having recognised that the value-for-money measure of cost-effectiveness does not deliver access to cancer medicines in line with community or political expectations, had established interim access measures while continuing the search for a better system. Mr Timothy James told the committee:

I think it is fair to say, without reflecting too much on the public policy decisions of other governments, that in both the UK and Canada they saw that a one-size-fits-all approach did not work and that, indeed, that approach was failing cancer sufferers. Hence, they determined that there should be a dedicated, specifically focused, resourced part of their system and a particular capacity to enhance access to cancer medicines. We believe that sort of focus, the resourcing, the consideration of processes and decisions, the engagement of stakeholders in a range of respects obviously to have that greater enhanced focus upon cancer medicines and the particular challenges and indeed opportunities around cancer medicines, that is obviously something we would welcome in the local context.<sup>7</sup>

5.8 A number of submitters advocated the establishment of an interim access scheme within or closely aligned with the existing PBAC/PBS mechanisms.<sup>8</sup> The Tasmanian Government was not supportive of the establishment of a separate funding stream specifically for cancer medicines outside the PBAC process, stating that it is preferable for the PBAC process to be streamlined and tailored where possible.<sup>9</sup>

---

5 *Submission 117*, p. 4.

6 *Submission 188*, p. 4.

7 Mr Timothy James, *Committee Hansard*, p. 6.

8 See for example: Rare Cancers Australia, *Submission 92*, pp 10 – 12; Unicorn Foundation, *Submission 130*, p. 4; Cancer Drugs Alliance (CDA), *Submission 53*, p. 1; Tasmanian Government, *Submission 188*, pp 4-5.

9 *Submission 188*, p. 5.

---

5.9 The Cancer Drugs Alliance (CDA) proposed the establishment of an interim access scheme while 'the Government commences the process of PBS modernisation'.<sup>10</sup> Like the Tasmanian Government, the CDA considers that an interim access scheme should be established within the existing PBAC/PBS mechanisms and should:

- be designed to provide access to medicines between Therapeutic Goods Administration (TGA) registration and PBS reimbursement;
- time-limited and operate until revised PBS/PBAC measures have been implemented;
- operate within and in parallel to the existing PBS system, which would continue to execute its obligations to approve and fund cancer drugs based on the existing framework;
- include clinically driven guidelines for listing and de-listing drugs;
- include clear guidelines around acceptable pricing taking account of issues faced by all stakeholders; and
- be supported by the establishment of a database of outcomes following the use of chemotherapy and targeted medicines.<sup>11</sup>

5.10 Some submitters saw benefits in establishing an interim fund to provide expedited access to treatments for rare and less common cancers, including treatments already listed on the PBS for other indications.<sup>12</sup> Rare Cancers Australia (RCA) proposed that an interim access scheme should be administered by the Department of Health (DOH) for the interim approval of medicines not listed on the PBS. RCA proposed the following process:

- applications to be made by a suitably qualified clinician; and
- for indications considered to be rare or less common and for which the company will not be making a submission to the PBAC for that indication; and
- reviewed by a panel of clinicians and patient advocates who would provide feedback to the DOH on each drug's safety, efficacy and potential value; and
- that the supply of approved medicines could be subject to a range of other considerations, including a limited time period; a price agreed to between

---

10 CDA, *Improving Access to Cancer Medicines*, White Paper, March 2015, p. 25.

11 CDA, *Improving Access to Cancer Medicines*, White Paper, March 2015, p. 25. In March 2014, the CDA hosted a forum of stakeholders from across the cancer community to discuss and build strategies to support both short and long-term access to cancer medicines. The CDA Forum identified five priority areas for improving access to cancer medicines for Australian patients and established work-streams to address these. The outcomes of these work-streams are set out in the White Paper; [www.cancerdrugsalliance.org.au](http://www.cancerdrugsalliance.org.au) (accessed 7 September 2015)

12 See for example, Mr Andrew Warden, *Submission 7*, p. 2.

DOH and the manufacturer, and an agreement with regard to any outstanding amounts paid to the company in the event the drug is listed by the PBAC.

5.11 RCA also stated that it may be necessary to implement some form of time-limit penalty to ensure that applications are considered in a timely manner.<sup>13</sup>

5.12 However, some submitters cautioned against the establishment of a dedicated cancer fund, noting that overseas experience suggests that such funds have not necessarily alleviated issues around cost and access to high cost medicines.<sup>14</sup> For example, Cancer Council of Australia and Clinical Oncology Society of Australia (CCA/COSA) noted that while the CDF had improved access to new listings, the fund was over budget and has been criticised for not addressing the issue of price negotiation with manufacturers.<sup>15</sup>

5.13 The Society of Hospital Pharmacists of Australia (SHPA) was also not supportive of the establishment of alternate funding programs for specific patient groups such as oncology:

SHPA believes that the current standard assessment and approval systems are essential to ensuring the safety and cost effectiveness of medicines funded by public monies or through the PBS. We do not believe that these evidence-based decision-making principles should be compromised for any patient group.

5.14 SHPA further stated

...if the Australian government was to expand the range of medicines funded outside the PBS, the limitations and capacity of systems such as the Australian Life Saving Drugs Program and the Cancer Drugs Fund in the United Kingdom must be examined in detail.<sup>16</sup>

5.15 In its submission, SHPA noted a range of concerns about the CDF, including:

- the fund's use of less stringent approval processes compared to the standard NICE process;
- a tendency for the price paid by the fund to result in the UK paying a higher price for cancer medicines than most European countries;
- the diversion of funds away from potential treatment alternatives;
- overspending has resulted in only 59 of 84 currently listed medicines being funded in 2015-16; and

---

13 *Submission 92*, pp 10-11.

14 See for example, Pharmaceutical Society of Australia, *Submission 176*, p. 6; Dr Agnes Vitry, *Submission 128*, p. 8.

15 *Submission 145*, p. 10.

16 *Submission 112*, p. 2.

- 
- the CDF has undermined the NICE and the principle of evidence-based decision making.<sup>17</sup>

5.16 Roche Products also considered that the CDF has its limitations, 'principally the lack of an acceptable method of prioritising medicines to list, and the perception of discrimination against other high-burden health conditions'. Roche Products submitted that it supports systemic reform that will improve access for all specialised medicines, including cancer. However, Roche Products noted that, as reform would take time, the government should consider models for providing interim access to new cancer therapies.<sup>18</sup>

5.17 Ms Simone Leydon from the Unicorn Foundation told the committee that, notwithstanding the difficulties encountered with the CDF, there was merit in considering such a scheme as an interim measure:

There have been different mistakes probably made with that, but the essence of it is that it provides an interim model—and we would stress that these are interim models—while we look at reviving or changing some parts of the PBS. They would not be there for the long term, which, unfortunately, I think has happened overseas. And they would be more strictly controlled. So, again, there would have to be some sort of restrictions put on what drugs could go in there and how much and that sort of thing.<sup>19</sup>

#### *The Cancer Drugs Fund*

5.18 A significant number of submitters suggested that an interim specialist fund could be modelled on the United Kingdom's CDF.

5.19 As noted above, the CDF was established in response to criticism over the timeliness of access to new cancer medicines, to fund access to cancer drug treatments which:

- were not approved by NICE due to insufficient cost effectiveness;
- had not yet received final NICE guidance;
- were for rare cancer licensed drug indications not selected for NICE appraisal; or
- were planned to be used off label (and therefore could not be assessed by NICE).

5.20 The current operating model for the scheme is due to end on 31 March 2016. Initial funding for the CDF was originally capped at £200 million per year. In 2013-14, the fund was overspent by £30.5 million. The UK government announced that it would increase the CDF budget to £280 million in 2014-15 (and to £340 million

---

17 *Submission 112*, pp 7-8.

18 *Submission 114*, p. 16.

19 *Committee Hansard*, p. 45.

in 2015-16), and that the list of drugs on the CDF would be reviewed, with a focus on 'value for money'.<sup>20</sup>

5.21 NHS England told the committee that while the CDF had substantially increased access to cancer drugs, particularly for the treatment of rare cancers or rare cancer indications which had not been assessed by NICE, the CDF has had unintended consequences:

An unintended consequence of the CDF has been to initially reduce the incentive for drug manufacturers to reduce the prices of their drugs to make their drugs cost effective via a NICE appraisal. However, the recent incorporation of drug cost when added to an assessment of clinical impact into the overall CDF decision-making process has restored this need. The consequence of the setting up of the CDF has also been to have an ever increasing number of CDF drugs without final decisions as to whether they should be in baseline commissioning or not and only definitively making such decisions when the CDF funding envelop is threatened. An additional observation has been the recent trend for drugs to be licensed on relatively preliminary data which creates much uncertainty in NICE's assessment of cost effectiveness. This phenomenon is an additional factor in explaining the recent higher rate of NICE negative recommendations.<sup>21</sup>

5.22 In December 2014, a CDF Working Party was established, comprised of NHS England, NICE, the Department of Health and representatives of the pharmaceutical industry and cancer charities, to co-produce a proposal for a framework for the long-term sustainable evaluation and commissioning of cancer drugs. The framework is expected to be ready for public consultation during 'the summer of 2015' and for implementation from April 2016.<sup>22</sup>

5.23 One of the potential solutions being considered by the Working Party is a 'managed access' approach to the CDF 'with clear entry and exit criteria and procedures which would be run jointly between NHS England and NICE.

5.24 NHS England told the committee:

The proposal recognises NICE's strength in the determination of overall clinical and cost effectiveness and the ability of NHS England to produce robust clinical commissioning policies for rare or off label cancer drugs. Both of these routes of assessment in the new process will incorporate an initial consideration of clinical promise and thus prioritisation of drugs which deliver the most important, favourable and meaningful outcomes.<sup>23</sup>

5.25 NHS England said the managed access approach is considered to allow the potential for immediate access to funding on licensing and the collection of further clinical outcome data if the NICE technology appraisal concludes that longer term

---

20 DOH, *Submission 197*, p. 17.

21 NHS England, *Answer to written question on notice*, received 15 June 2015, p. [2]

22 NHS England, *Answer to written question on notice*, received 15 June 2015, p. [2]

23 NHS England, *Answer to written question on notice*, received 15 June 2015, p. [2]

---

data are required. NHS England describes this as a 'Commissioning through Evaluation' model.<sup>24</sup>

### Managed access models

5.26 A number of submitters proposed the adoption of a model for managed access to medicines as a means of addressing concerns regarding evidence gaps and timeliness in the PBAC assessment process.<sup>25</sup> For example, Roche Products expressed support for the more dynamic approach to Health Technology Assessment (HTA) that a managed access scheme may provide:

Currently, under managed entry, an initial subsidy is provided at a price justified by the existing data, pending the submission of more conclusive evidence. Roche consider that the initial price must reflect the value of the product and be in step with launch prices in other developed markets. The totality of available evidence needs to be considered, and subsequent evidence collection must be fit-for-purpose (i.e. address the identified uncertainties).<sup>26</sup>

5.27 The committee notes that Australia has had a framework for a Managed Entry Scheme (MES) since 2011. Under this framework, the PBAC has had the ability to recommend PBS coverage for a drug at a price justified by the existing evidence, pending submission of more conclusive evidence of cost-effectiveness.<sup>27</sup> The expectation this measure is that the price of the drug could subsequently be adjusted, either up or down.<sup>28</sup>

5.28 The PBAC provided the following explanation of how managed entry works:

In managed entry, a provisional price for the drug is set on the basis of the sponsor's estimate of effectiveness and toxicity, while data on outcomes are systematically collected from patients and prescribing doctors. In this way the clinical risk of lack of benefit and potential for harm are countered, because those outcomes are detected early because national data are collected. The financial risks associated with PBS-listing a drug whose effectiveness is uncertain, but which the PBAC believes is not likely to be cost-effective at the sponsors' preferred price, are shared between sponsor and government because the sponsor agrees to repay money if the drug is less effective in actual use than was predicted.<sup>29</sup>

---

24 NHS England, *Answer to written question on notice*, received 15 June 2015, p. [2]

25 See for example: Janssen-Cilag Pty Ltd, *Submission 140*, p. 10.

26 *Submission 114*, p. 13.

27 DOH, The Pharmaceutical Benefits Scheme, *Framework for the introduction of a Managed Entry Scheme for submissions to the Pharmaceutical Benefits Advisory Committee*, [www.pbs.gov.au](http://www.pbs.gov.au) (accessed 10 June 2015).

28 See for example: Dr Katherine Nielsen, Director, Research and Advocacy, Ovarian Cancer Australia, *Committee Hansard*, p. 10.

29 *Submission 196*, p. 14.

5.29 However, evidence to the committee was that the MES had not been enthusiastically embraced by industry. The Leukaemia Foundation of Australia (LFA) noted the limited use made of the MES to date and that the scheme is currently under review:

The PBAC requires greater flexibility to list new drugs, such as TGA listed orphan drugs, which have limited clinical data due to small patient cohorts. The Managed Access Scheme was introduced as a solution to this in 2011. However, the program has not been a success and is under review. A less restrictive scheme is essential, allowing conditional listing with further data to be supplied to demonstrate clinical benefit.<sup>30</sup>

5.30 DOH told the committee that changes, such as the MES and 'pay for performance', are slowly being taken up by industry 'as their value is recognised'.<sup>31</sup> DOH submitted that:

...the increasingly expensive price of [cancer medicines] represents marginal value and that it is difficult to justify continuing acceptance of high costs for treatments that offer very small benefit. It is vital that PBS pricing policies continue to put pressure on medicine pricing and further consideration of 'pay for performance' (ensuring that the price reflects available evidence of the health benefit) is also warranted.<sup>32</sup>

5.31 DOH further stated:

The PBS has adopted innovative pricing models to provide access to new drugs whilst also supporting the development of a stronger evidence base. For example, the existing 'managed access' approach is being reinvigorated to provide options for medicines that are used to treat rare cancers by allowing a phased evaluation and listing, linked to progressive payments. Earlier access than would otherwise be obtained could be granted, where safe to do so, for use in those patients who have no other treatment options. The health outcomes would be tracked and reviewed, with approval for broader use only once sufficient evidence of effectiveness becomes available.<sup>33</sup>

5.32 MA told the committee that the industry is open to working with 'public policy makers' on the development of the MES scheme, but noted:

There is a balance to be struck between getting access to patients who need those medicines most as early as possible and being prepared to submit to requirements for both clinical and cost effectiveness.<sup>34</sup>

5.33 Notwithstanding the limited use made of the MES to date, submitters noted the potential for such an approach to address concerns regarding the collection of

---

30 *Submission 123*, p. 7.

31 *Submission 197*, p. 1.

32 *Submission 197*, p. 21.

33 *Submission 197*, p. 20.

34 Mr Timothy James, Chief Executive Officer, Medicines Australia, *Committee Hansard*, p. 8.



---

clinical data and the consideration of broader social and economic factors, by capturing 'real world' experience of new cancer treatments. Mr Richard Vines, Executive Chair, RCA told the committee that the solution requires a bit of flexibility:

I go back to where you were talking about 'once a submission is lodged'. Once it is registered and we know there is going to be a submission, and the TGA says it is safe and the pharmaceutical company is definitely making a submission, from that point on, if we can run a managed entry scheme after PBAC consideration, we ought to be able to put something in place beforehand, not that corrupts the PBS process—it has served us well for a very long time—but allows it to go through the one to two years, if that is necessary, and patients still have access. The issue is not the delay; the issue is the patients who do not get it while the delay takes place.<sup>35</sup>

5.34 Mr John Cannings, who appeared in a private capacity, told the committee:

The issues are that currently, under the present guidelines and PBAC rules, PBAC are not able to take into account some of both social factors and economic factors in their determination around cost-effectiveness. Those rules need to be modernised to allow earlier access. Part of that could be through a managed entry scheme arrangement, where this real-world evidence is then obtained, accumulated and put back into the system for all cancer sufferers.<sup>36</sup>

5.35 The PBAC noted that there are a number of issues that need to be considered in formulation recommendations based on a managed entry approach:

- the PBAC must have confidence that the clinical data provided at the initial application shows evidence of likely benefit of treatment to patients;
- the sponsor should have additional studies in progress that will potentially confirm this benefit and allow accurate assessment of the size of the benefit over existing treatments;
- alternatively, the sponsor needs to be prepared to collect data from Australian patients to establish the benefits, harms and costs of treatment. Clinicians and patients therefore need to agree to have such data collected; the committee notes that this raises issues of privacy that are beyond its remit, but that optimal implementation of managed entry may require legislative change;
- the sponsor needs to propose a price that is potentially cost-effective, on the basis of the data available at the time of PBAC consideration; and
- the sponsor and DOH need to execute a contract to ensure all of these issues are agreed, as well as a strategy for delisting the product and/or recovering excess payments if the hoped-for benefits are not confirmed. This process,

---

35 *Committee Hansard*, p. 40.

36 *Committee Hansard*, p. 44.

including the fact the drug may be delisted, need to be very clearly communicated to patients.<sup>37</sup>

5.36 The committee notes that some of the PBAC's more recent recommendations have been on the basis of a MES. For example, in announcing the listing of the drug Crizotinib, used to treat anaplastic lymphoma kinase – positive non-small cell lung cancer, on 1 July 2015, the Minister for Health, the Hon Sussan Ley MP, stated that listing through a MES would speed up access for patients with the highest need for treatment.<sup>38</sup> In chapter 4, the committee noted evidence from RCA and Mr Cannings regarding delays in the listing of Crizotinib.<sup>39</sup> The PBAC recommended listing of Crizotinib following its November 2014 meeting, having first considered an application for its listing at its November 2013 meeting. The drug was registered by the TGA on 27 September 2013.<sup>40</sup>

5.37 In evidence to the committee, Dr Suzanne Hill, former Chair of the PBAC, indicated that the PBAC is continuing to make recommendations for listing in certain circumstances on the basis of managed entry, while the future application of managed entry is being considered:

In terms of actual managed entry schemes, the committee has recommended already a couple of products with listings that are effectively managed entry. One was ipilimumab for melanoma, nearly 18 months ago, where the agreement was that the price would be set on the basis of the data that was available to the committee at the time for the cost-effectiveness evaluation; that survival data would be collected; and then, when the two years survival data was available, that the cost-effectiveness would be re-evaluated on the basis of the survival and toxicity data seen in the real world. Subsequently, the committee has made another couple of recommendations from November in a similar vein.

So there are two parts to it. There are the recommendations that are already coming out of the committee that are effectively managed access or managed entry schemes, and then there is some more general discussion of managed entry or managed access.<sup>41</sup>

5.38 The committee notes that the discussion around the development of managed access mechanisms appears to be centred in the work of the Access to Medicines

---

37 *Submission 196*, p. 14.

38 The Hon Sussan Ley MP, Minister for Health, Media Release, New breast and lung cancer drugs available from today, 1 July 2015.

39 See paragraphs 4.56 and 4.70 respectively.

40 PBS, Crizotinib; 200 mg capsule, 60 and 250 mg capsule, 60; Xalkori®, *Public Summary Document – November 2014 PBAC Meeting*, p. 2, [www.pbs.gov.au](http://www.pbs.gov.au) (accessed 7 September 2015).

41 *Committee Hansard*, pp 75-76.

Working Group (AMWG).<sup>42</sup> Since June 2014, the Managed Access Programme Sub-Group of AMWG has been developing a framework for a Managed Access Programme. At its December 2014 meeting the AMWG noted substantive progress on the project and anticipated broader review of the framework early in 2015. The PBAC reviewed the draft framework at its March 2015 meeting.<sup>43</sup> It is not clear if broader consultation with clinicians and consumers is contemplated in finalising the draft framework.

### *Alternate models*

5.39 The committee notes evidence emphasising that managed access is only one possible avenue for addressing demand for early access to new medicines. Submitters noted a managed access scheme would work well alongside other mechanisms currently provided for within the PBS such as risk sharing between the Australian Government and the sponsor of a medicine.<sup>44</sup>

5.40 Submitters also noted that the long-term challenge of funding subsidised access to cancer medicines requires the consideration of a range of new approaches. The committee received evidence suggesting that regulatory models used in other policy domains may serve as useful models for addressing challenges created by rapid scientific advances in cancer treatment. The committee received evidence regarding two such models: the licensing of oncology medicines and social impact bonds.

### *Risk sharing agreements*

5.41 The committee notes risk sharing agreements are intended to help maintain the appropriateness and cost-effectiveness of listed medicines.<sup>45</sup>

5.42 CCA/COSA expressed support for use of risk-sharing arrangements as a means of incorporating the use of surrogate endpoints into the evaluation of cancer drugs:

As recommended against ToR a), a scheme based on surrogate endpoints (also known as performance-based, risk sharing arrangements) could be implemented in Australia. The scheme could involve new cancer drugs being submitted for funding based on surrogate endpoints (such as progression-free survival) with an upfront agreement (not subject to appeal)

- 
- 42 The Access to Medicines Working Group was formed by DOH and MA as part of the PBS reforms announced in 2006 to assist them to work together more effectively and to consider issues regarding timely and appropriate access to new medicines for the PBS. The membership of the working group comprises DOH and MA, but has agreed to consult with other stakeholders when issues may impact on them. See: DOH, Access to Medicines Working Group, [www.health.gov.au](http://www.health.gov.au). (accessed 15 September 2015).
- 43 DOH, The Pharmaceutical Benefits Scheme, March 2015 PBAC Minutes for the Managed Access Program Framework, [www.pbs.gov.au](http://www.pbs.gov.au) (accessed 15 September 2015).
- 44 See for example: Ovarian Cancer Australia, *Answer to question on notice*, 20 April 2015 (Received 22 May 2015).
- 45 DOH, The Pharmaceutical Benefits Scheme, *Step 7, Entering agreements to share risk*, [www.pbs.gov.au](http://www.pbs.gov.au) (accessed 15 September 2015).

that funding would be reduced if the drug, in post-market evaluations, did not realise a major endpoint such as overall survival or improved quality of life.

Post-marketing surveillance under this type of scheme would need to be strictly conducted, as the earlier a drug is marketed, the greater the risk of uncovering unusual or adverse effects.<sup>46</sup>

5.43 However, Roche Products submitted that while risk-sharing agreements are aimed at reducing listing delays following a positive PBAC recommendation, they are frequently one-sided and may impose requirements that are not based on clinical best-practice but simply reducing financial costs to government beyond what is required for cost-effectiveness. However, Roche Products recommended that industry and government should work together to identify opportunities to further streamline listing processes.<sup>47</sup>

5.44 Merck Sharp and Dohme (MSD) also stated that the reason for the poor take up of the original MES was the perception that 'all risk in participating in the scheme would be borne by the companies, with little hope of price increases even if conclusive evidence was forthcoming'. MSD expressed hope that the development of a new framework through the AMWG would formalise a more effective way of giving patients access to new products.<sup>48</sup>

#### *Licensing innovative oncology medicines*

5.45 RCA questioned whether the current approach of treating medicines as products and seeking approval on an individual product basis remains an appropriate model. RCA suggested that treating innovative medicines as intellectual property and applying a 'service' model, similar to that used for software, music and film:

The proposition is that, in order to address the failings and delays of the current system and to avoid the future capacity issues that seem likely, we look at the model of "Medicines as a service". In other words instead of pricing and costing each tablet or ampule as a separate exercise we examine the possibility of licensed usage for a medicine.<sup>49</sup>

5.46 RCA states that this type of funding model has the capacity to deliver benefits in terms of certainty, flexibility and simplicity.<sup>50</sup>

#### *Social impact bonds*

5.47 The CDA White Paper proposed further consideration of social impact bonds (SIBs) as means of providing incentives from investments in cost-saving preventive services. CDA stated that SIBs can ensure that public funding goes only to

---

46 *Submission 145*, p. 10.

47 *Submission 114*, p. 15.

48 *Submission 120*, pp 4-5.

49 *Submission 92*, p [14]

50 *Submission 92*, p. [14].

interventions that demonstrate their impact through rigorous outcome-based performance measures.

Under the most common social impact bond model, the government contracts with a private sector intermediary to obtain social services. The government pays the intermediary entirely or almost entirely based upon achievement of performance targets.<sup>51</sup>

5.48 The CDA considered that SIBs offer innovative solutions for funding health initiatives and could have long-term benefits beyond the funding of access to cancer medicines. Australia's first SIB is the Newpin Social Benefit Bond, funded by the NSW Government.<sup>52</sup> The CDA states that in proposing consideration of alternate access models such as SIBs, it is not attempting to design alternative funding models external to the PBS. Its intention is to identify models that might 'relieve pressure on already constrained resources, while ensuring we improve access for Australian patients'.<sup>53</sup>

### **Improved monitoring and data collection**

5.49 Evidence to the committee underscored the importance of improved data collection and greater integration of existing data bases to improving the speed with which cancer drugs are assessed for registration and reimbursement and the level of information available to assist clinicians. Evidence to the committee noted that the success of managed access programs for new medicines is dependent on accurate data capture systems.<sup>54</sup>

### ***Post marketing surveillance***

5.50 Submitters stressed that it is important to continue to monitor clinical and cost effectiveness of all medicines after they are listed on the PBS and recommended the application of greater use of post-market review processes.<sup>55</sup> The committee heard that currently little is known about patterns of use, patient outcomes or safety following the grant of marketing approval.<sup>56</sup>

---

51 CDA White Paper 2015, p. 27.

52 Newpin Social Benefit Bond is a long-term, centre-based, intensive support program that works with families to improve parenting so children can live safely at home. The CDA White Paper states that in August 2014 it was reported that Newpin's maiden return to investors had a yield of 7.5 per cent on the \$7 million bond.

53 CDA White Paper 2015, footnote d, p. 26.

54 See for example: Delivering Affordable Cancer Care: A Value Challenge to Health Systems, Report of the World Innovation Summit for Health (WISH) Delivering Affordable Cancer Care Forum 2015, cited in DOH, *Submission 197*, p. 16.

55 See for example, Cancer Voices Australia, *Submission 49*, p. 1; MSD, *Submission 120b*, pp 5-6; CCA/COSA, *Submission 145*, p. 10; Cancer Voices SA, *Submission 150*, p. 2

56 See for example: CDA White Paper, p. 19.

5.51 CCA/COSA told the committee that while they consider there is an excess level of rigidity in the pre-market assessment of medicines, the same rigour is not applied to assessing the efficacy and cost-effectiveness of medicines once listed:

The absence of rigorous, ongoing post-listing review can lead to unnecessary expenditure and suboptimal use of listed medicines. Greater rigour in post-market review is a potential cost offset that could allow for the listing of new medicines which, while vitally important to a comparatively small number of patients, do not currently meet cost-effectiveness criteria. Greater rigour in post-market evaluation would also be a necessary tool for accepting surrogate endpoints other than disease-free survival as indicators of efficacy when assessing new PBAC applications – as recommended in response to the problems of timing and delay.

Agreed future milestones could be monitored through regular post-market assessment using agreed, pre-determined reporting mechanisms. It would require a commitment from the sponsor to provide results of ongoing studies and greater monitoring of safety and efficacy post-market by the TGA.<sup>57</sup>

5.52 SHPA also expressed concern that under the current system of post-marketing surveillance products are reviewed in an ad hoc manner in response to stakeholder concerns about a particular product. SHPA recommended that all medicines funded through the PBS should be systematically and routinely assessed against the criteria that was initially used to approve the product for listing, as well as any newer, relevant evidence which has been published since the time of listing.<sup>58</sup>

5.53 COSA member Ms Suzanne Kirsas told the committee that improved post-market surveillance to ensure that listed medicines are continuing to provide value for money, could help to offset some of the costs associated with the listing of new medicines via more flexible evaluation processes.<sup>59</sup>

5.54 A system of post-market reviews was introduced following the 2011-12 budget to assist with improving the sustainability of the PBS. Post-market reviews provide a means of monitoring medicines in use to inform decision making at all levels throughout the medicine cycle, from registration to its use by consumers. The committee notes the post-market review program is intended to contribute to:

- improved patient safety through better understanding of adverse events and medicine-related harms;
- ensuring the ongoing viability of the PBS through targeted medicines usage and avoiding preventable wastage or inappropriate prescribing;
- a better understanding of medicines utilisation, to review intended clinical benefit and inform medicines evaluation processes;

---

57 *Submission 145*, p. 7.

58 SHPA, *Answer to question on notice*, 22 April 2015 (received 22 May 2015).

59 *Committee Hansard*, p. 52.

- ongoing cost-effectiveness, including through better management of clinical and economic uncertainty; and
- overall improvements to the quality of use of medicines and education for patients and prescribers.<sup>60</sup>

5.55 The committee notes that new guidance for post-market reviews of medicines listed on the PBS were announced in March 2015. The new guidance was produced by the AMWG and has been agreed by the pharmaceutical industry, MA and DOH.<sup>61</sup>

### ***Cancer registries***

5.56 Submitters noted that governments in other countries are increasingly recognising the value of a coordinated national approach to data collection to justify expenditure on cancer treatments, to provide a framework for earlier access to new treatments and drive improvements in the delivery of better outcomes for cancer patients.<sup>62</sup> Ms Carlene Todd of Roche Products said:

What we can learn from other countries though is around collection of clinical data in the real world. Italy and the Netherlands do this well. They have cancer registries in place and they can collect evidence in clinical practice over time for medicines.<sup>63</sup>

5.57 In its White Paper, CDA advocated the establishment of an Australian national chemotherapy registry (NCR) to enable identification of trends in clinical practice and patient outcomes. CDA noted that this information could be used to inform and improve the quality of care across the country:

The NCR's focus will be to improve patient outcomes by monitoring and improving quality of care. The main purpose of the data collection would be to:

- Monitor current relevant patient information and linking to medicine use and patient outcomes, including safety and efficacy;
- Report risk-adjusted benchmarked data with the purpose of improving quality of care and delivering optimal patient outcomes;
- Facilitate decision-making for access to new cancer medicines on the pharmaceutical benefits scheme (PBS);
- Provide a framework that would support earlier access to cancer therapies such as through managed entry schemes/managed access programs;
- Provide a framework for the collection of real world data to measure cost effectiveness in Australian clinical practice; and

---

60 DOH, The Pharmaceutical Benefits Scheme, *Post-Market Reviews of Pharmaceutical Benefits Scheme Subsidised Medicines*; [www.pbs.gov.au](http://www.pbs.gov.au) (accessed 15 September 2015)

61 The Hon Sussan Ley MP, Minister for Health, Media Release, *Positive progress and achievement from AMWG*, 18 March 2015.

62 CDA White Paper, p. 6.

63 *Committee Hansard*, p. 28.

- Monitor and report on delivery of equitable cancer care across Australia.<sup>64</sup>

5.58 MSD also supported the establishment of a NCR to continually assess the efficacy of medicines and to help demonstrate the value that medicines bring to society:

At present, despite significant expenditure on cancer medicines, no comprehensive picture exists of what happens to patients once they are placed on therapy, as no national database exists which captures this information. As well as empowering clinicians with information to improve cancer care, such data provides a framework to monitor real world cost effectiveness and to support Managed Access Schemes. This would enable the health community to continually monitor whether it is getting value for money out of its investment.<sup>65</sup>

5.59 However, the committee also heard that, before investing in a national clinical cancer registry, there is a need to address mechanisms for the collection of data on a national basis.

We do not have a national database. There are a lot of disparate registries. What the Medicines Australia submission talks about and some of the others as well is the need to link some of those registries together to make sure that they are talking to each other so we can access the data within those registry silos. That is a big issue at the moment, and something we should probably look at first before we invest in a national registry.<sup>66</sup>

### ***Greater integration of existing datasets***

5.60 Submitters noted that greater integration of existing datasets would provide a means of harnessing the potential of real time monitoring of outcomes, both from clinical trials and post-PBAC approval, to support managed entry and improved patient care. For example, COSA suggested the 'integration of post market surveillance and reporting to track cancer medicines introduced into the market early, as well as the ongoing effectiveness of approved medicines and technologies'.<sup>67</sup>

5.61 Dr Hill told the committee that more effective collection of clinical outcome data such as fact of death or adverse effects would be 'an enormous advance' in supporting the use of managed entry schemes:

Equally, being able to collect clinical outcomes such as fact of death or adverse effects more effectively than we can at the moment would be an enormous advance in trying to arrange what we have called managed entry schemes, where we need to try and monitor drugs that are made available to

---

64 CDA White Paper, pp 22- 23.

65 *Submission 120*, p.5.

66 Ms Carlene Todd, Director, Market Access and Pricing, Roche Products, *Committee Hansard*, p. 28.

67 COSA, *Answer to question on notice*, 20 April 2015 (received 22 May 2015),



---

the community early to make sure that the benefits and harms are the same as what we see in the clinical trials.<sup>68</sup>

5.62 In its submission to the inquiry, the PBAC emphasised the importance of linking data sets to support early access.<sup>69</sup> Professor Zalcborg of CDA also emphasised the merit in linking existing databases to enable a more comprehensive understanding of cancer drugs:

Clinical trial data is often incomplete at the time. For example, long-term survival issues are not available; the true toxicity or safety profile is not understood; we have heard about progression-free survival and overall survival; there may be interim end-points. So there is a need to get further data. The way to collect that data is to link existing databases—like Medicare, like PBAC, like the Australian Cancer Database—in a way that protects people's privacy but allows information that has already been collected and information that can be collected into a comprehensive picture about what these drugs are doing and what they are not doing.<sup>70</sup>

5.63 The Australian Cancer Database (ACD) is a data collection of all primary, malignant cancers diagnosed in Australia since 1982. The ACD is compiled by the Australian Institute of Health and Welfare (AIHW) from cancer data provided by state and territory cancer registries through the Australasian Association of Cancer Registries, which in turn receive information on cancer diagnoses from a variety of sources including: hospitals, pathology laboratories, radiotherapy centres and registries of births, deaths and marriages. Data from the ACD is used to report on national cancer statistics such as incidence, trends, projections, survival and prevalence. While the AIHW acts as custodian of state and territory registry data for the purposes of producing national cancer statistics, the cancer registries retain ownership of their jurisdiction's data at all times. The AIHW is able to make available a broad range of cancer statistics subject to a scientific and ethical review process.<sup>71</sup>

5.64 DOH noted that the increasing use of e-health records may also assist in data collection.<sup>72</sup>

5.65 LFA expressed concern that without access to good information some patients may be being offered treatments that are no longer considered to be 'best practice'. LFA submitted that Australia needs a centralised process for all drug access inquiries:

Without a national clinical cancer database, factually-based information about which therapies are best practice in the Australian community remains unknown. Therefore, therapy decisions are essentially educated

---

68 *Committee Hansard*, p. 75.

69 *Submission 196*, p. 15.

70 *Committee Hansard*, p. 64.

71 Australian Institute of Health and Welfare, *Australian Cancer Database (ACD)*, [www.aihw.gov.au](http://www.aihw.gov.au) (accessed 15 September 2015).

72 *Submission 197*, p. 16.

guesswork and doctor preference, and may be subjected to conflicts of interest.<sup>73</sup>

5.66 Professor Brendon Coventry of the Australian Melanoma Research Foundation also described the difficulty in accessing data:

It is really hard. In the melanoma foundation we have been trying to get the information about complete response rates, outcomes, stuff that you should be using to make your decision, your informed choices. We find it really hard to get. It is buried in papers, it is in tables, it is in supplementary information and so on. This is extremely difficult for an experienced clinician and a group of clinicians to do. The patient has almost no hope of doing it on their own, unless they have the experience. We are trying to distil that information, compare the studies, compare the trials, put the information on the table so they walk up to one source and get that information that they need to have at their fingertips in order to make an informed choice in the clinic when the clinician starts speaking to them.<sup>74</sup>

5.67 Mr Martin Ashdown, Research Fellow in the Faculty of Medicine at the University of Melbourne suggested that the AIHW might play a role in facilitating access to clinical data:

It would be very valuable if the Australian Institute of Health and Welfare could also collect survival and complete response data for different drug treatments and their associated costs to enable closer evaluation of clinical effectiveness for patients and clinicians, and especially to permit better informed choices.<sup>75</sup>

5.68 Leukaemia Foundation of Australia (LFA) noted the emergence of private pay-for-service models, such as Biogrid, which collects data from patients with a range of cancers and other health conditions that are being treated in a number of hospitals in Victoria, Tasmania, South Australia, ACT and Queensland. LFA stated that an example of the value of such clinical databases was 'the analysis of outcomes for patients with metastatic colorectal cancer' which had 'enabled differences in practice between centres to be identified, and practice reviewed and standardised to improve patient outcomes'.<sup>76</sup>

5.69 However, LFA argued that such databases need to be established on a national scale so that they can form part of everyday cancer care delivery and not be 'available just to the small number of patients who find out about it, either through their health provider, or online'.<sup>77</sup> LFA noted that a national clinical database could also facilitate

---

73 LFA, *Answer to question on notice*, 22 April 2015, (received 22 May 2015), p. 3.

74 *Committee Hansard*, p. 17.

75 *Committee Hansard*, p. 10.

76 LFA, *Answer to question on notice*, 22 April 2015, (received 22 May 2015), p. 3. LFA referred the committee to the Biogrid Annual Report 2013-14: [http://biogrid.blob.core.windows.net/assets/uploads/files/BioGrid\\_Annual\\_Report\\_2013\\_2014\\_v2.pdf](http://biogrid.blob.core.windows.net/assets/uploads/files/BioGrid_Annual_Report_2013_2014_v2.pdf) (accessed 15 September 2015).

77 LFA, *Answer to question on notice*, 22 April 2015, (received 22 May 2015), pp 2- 3.

---

the review of changes in indication, extending approval to other cancers with the same genetic mutations and survival benefits, by providing the TGA and the PBAC with access to extensive data, over and above that provided by the drug sponsor.<sup>78</sup>

### ***Capturing data from off label use of medicines***

5.70 Submitters also noted the importance of capturing information about outcomes from off label use of cancer drugs. For example, Cancer Voices Australia recommended the use of post-marketing surveillance of real-life use of medicines, not just in clinical trials, to assess cancer drugs' effectiveness and impact on quality of life.<sup>79</sup>

5.71 Cancer Voices SA submitted:

We need routinely collected comprehensive, high quality data to monitor outcomes of all cancer patients. This data should be analysed and reviewed to ascertain the effectiveness of treatments, particularly high cost treatments. We need to be able to assess cancer drugs' effectiveness and impact on quality of life, not just in clinical trials, but through comprehensive post marketing surveillance. We are missing the information about the outcomes from all those who access these high cost drugs outside of clinical trial situations (eg via family and community fundraising) or special access schemes. We need to ascertain if the expected benefits are achieved when these drugs are used outside the clinical trial population.<sup>80</sup>

5.72 Ms Michelle Stewart of Cure Brain Cancer Foundation told the committee:

People are taking lots of different things and none of that data is being collected. For the larger group of patients, there is little benefit. We believe we could capture this off-label use, which is happening anyway, through a registry. Patients would then disclose what they are taking and we could track their responses on those treatments.

Clinical trials are set up for a good reason and that is to gain evidence for using a certain treatment. If you start using off label you must make sure that you are collecting the data as well.<sup>81</sup>

### **Committee view**

5.73 The committee notes strong support for the introduction of an interim specialist cancer drug fund, pending review of the current system for listing medicines on the PBS and the examination of other models for providing expedited access to medicines. Evidence to the inquiry is that the introduction of specialist schemes overseas has resulted in both faster approval times and the availability of a greater range of medicines to cancer patients. However, the committee notes concerns, both in

---

78 *Submission 123*, p. 10

79 *Submission 49*, p. 2.

80 *Submission 150*, p. 2.

81 *Committee Hansard*, p. 18.

Australia and overseas, that existing specialist schemes have not necessarily alleviated issues around cost and access to cancer medicines and that such funds have the potential to undermine the rigour of existing evaluation processes.

5.74 Evidence to the committee has emphasised the potential for a managed access program to address concerns regarding the availability of clinical data to demonstrate the case for listing medicines on the PBS without undermining the PBAC process. However, the committee notes the importance of effective monitoring of both clinical and cost effectiveness after PBS listing, both as a means of improving patient outcomes and as a basis for delisting medicines where appropriate.

5.75 Finally, the committee notes the importance of effective collection of clinical data and the merit in linking existing databases to enable more comprehensive analysis of the benefits of cancer medicines by the PBAC and clinicians and to support best practice in patient care.