The development of funding recommendations for health technologies at the state level: a South Australian case study

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Complete List of Authors:	Lambert, Robyn; South Australia Department for Health and Ageing, Medicines and Technology Programs; Royal Australasian College of Surgeons South Australia, Research and Audit Carter, Drew; University of Adelaide Adelaide Health Technology Assessment, School of Public Health, University of Adelaide Burgess, Naomi; South Australia Department for Health and Ageing, Medicines and Technology Programs and Out of Hospital Pharmacy Services HAJI ALI AFZALI, HOSSEIN; The University of Adelaide, Public Health;
Keywords:	resource allocation, decision making, health priorities, organisational case studies, health services research

Author biographies

Robyn Lambert

Robyn has recently completed a Masters in Public Health from the University of Adelaide and has worked in the Field of Health Technology Assessment for many years. Robyn has a keen interest in the processes and mechanisms that underpin resource allocation in health care.

Drew Carter

Drew Carter is a moral philosopher and health policy researcher. He applies insights made by Ludwig Wittgenstein to illuminate bioethical issues, especially relating to resource allocation. He has published on assisted reproductive technology and pain. His current research focuses on intensive care triage and the managed entry of health technologies

Naomi Burgess

Naomi Burgess has many years' experience in senior management, policy, research and teaching roles within the public hospital, state government and university settings. Her interests are in the safety, quality and cost effective use of health technologies and in systems for monitoring health outcomes to promote optimal patient care with a focus on the application of decision making in the real world setting

Hossein Haji Ali Afzali

Dr Hossein Afzali is a Senior Research Fellow with expertise in the economic evaluation of health technologies with a focus on the application of decision analytic modelling techniques. Dr Afzali co-leads the Adelaide Health Economics Research Group with expertise in all components of the economic evaluation of health care interventions

Title: The development of funding recommendations for health technologies at the state level: a South Australian case study

Short title: The development of funding recommendations for health technologies in South Australia

Robyn Lambert[1, 2]

Drew Carter[3]

Naomi Burgess[1]

Hossein Haji Ali Afzali[4]

Affiliations:

(1) SA Health, Adelaide, Australia

(2) Royal Australasian College of Surgeons, Adelaide, Australia

(3) Adelaide Health Technology Assessment, School of Public Health, University of Adelaide, Adelaide, Australia

(4) School of Public Health, University of Adelaide, Adelaide, Australia

Corresponding author: Robyn Lambert

Email: robynlbt@gmail.com

Abstract

Objectives: State governments often face capped budgets that can restrict expenditure on health technologies and their evaluation, yet many technologies are introduced to practice through state-funded institutions such as hospitals, rather than through national evaluation mechanisms. This research aimed to identify the criteria, evidence and standards used by South Australian committee members to recommend funding for high-cost health technologies.

Methods: We undertook eight semi-structured interviews and two meeting observations with members of state-wide committees that have a mandate to consider the safety, effectiveness and cost-effectiveness of high-cost health technologies.

Results: Safety and effectiveness were fundamental criteria for decision makers, who were also concerned with increasing consistency in care and equitable access to technologies. Committee members often consider evidence that is limited in quantity and quality; however, they perceive evaluations to be rigorous and sufficient for decision making. Precise standards for safety, effective and cost-effectiveness could not be identified.

Conclusions: Consideration of new technologies at the state level is grounded in the desire to improve health outcomes and equity of access for patients. High quality evidence is often limited. The impact funding decisions have on population health is unclear due to limited use of cost-effectiveness analysis and unclear cost-effectiveness standards.

Keywords

Resource allocation, decision making, health priorities, organisational case studies, health care rationing, health services research

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Conflicts of interest

Robyn Lambert conducted much of this research toward the completion of her Master of Public Health thesis, under the supervision of Drew Carter and Hossein Haji Ali Afzali. After completing her thesis, Robyn Lambert gained employment at SA Health. Robyn Lambert and Naomi Burgess work at SA Health in connection with the committees studied.

Introduction

Health Technology Assessment (HTA) is the field of inquiry dedicated to the systematic assessment of the value of health technologies. Many countries have established national processes that use HTA as a component of an evaluation framework when making public funding decisions. In Australia, for example, the Pharmaceutical Benefits Advisory Committee (PBAC) and the Medical Services Advisory Committee (MSAC) make recommendations to the federal government regarding the public funding of pharmaceuticals and medical services and procedures, respectively. The recommendations made by these committees are mainly based on evidence on the safety, effectiveness and cost-effectiveness of the technology under consideration.

Conceptually, national bodies such as PBAC and MSAC must first determine what factors *(criteria)* are important in ascertaining the value of a technology. They must then consider information *(evidence)* on how well the technology performs with respect to those criteria. Clearly, however, evidence on its own is not enough to inform funding decisions, and *standards* are required to judge when a technology is good *enough* to be recommended for funding.1,2 For example, when considering economic evidence, decision makers may apply a standard in the form of a threshold of cost-effectiveness representing the additional cost imposed on the health system (if a new technology is used instead of the comparator) to forgo one unit of the health outcome (e.g. quality-adjusted life-year).2

MSAC and PBAC have established criteria and describe the types of evidence that inform decision making, with technical guidelines setting out submission requirements for safety, effectiveness and cost-effectiveness.3,4 These committees also consider other important factors such as equity.5 However, standards used by these committees have not been explicitly acknowledged in their guidelines,6 though an implicit cost-effectiveness standard, for instance, has been inferred from past PBAC decisions 7 and can be glimpsed in recommendations citing “unacceptably high” cost-effectiveness profiles.2

Much less is published about the criteria, evidence and standards that inform decision making at the sub-national level in Australia and, indeed, at comparable jurisdictional levels internationally. Decision makers at the local (e.g. Local Health Network) and state level frequently receive requests for the coverage of health technologies which have been approved for marketing by the Therapeutic Goods Administration (TGA), but which have not been evaluated at the national level.8 Some technologies may never be submitted by manufacturers for consideration at the national level due to the costs involved in submission, long timeframes for assessment, or insufficient evidence to inform a national evaluation (as in the case of some rare medical conditions). Such technologies represent a challenge for all states, but there is no comprehensive interjurisdictional approach to their funding.9,10 This is particularly important when making decisions at meso or micro-levels, since they often face greater budget constraints than national funding bodies (e.g. they may have capped budgets).11

A 2011 review of formulary applications to Australian paediatric hospital Drug and Therapeutics Committees (DTCs) found that few applications were supported by high-level evidence, despite high approval rates.12 The review noted substantial variability, overlap and incomplete supporting documentation across institutions, and contrasted these findings with the rigorous process for listing drugs for national subsidy.12 The review indicated that, at

least with respect to medicines for paediatric patients, about which evidence can be especially difficult to generate for ethical reasons, the national and sub-national approaches to evaluating health technologies are dissimilar. A complete picture of the dissimilarities is lacking, however, since research examining decision making in Australia at sub-national levels is limited in volume and has overwhelmingly been conducted in the Australian state of New South Wales (NSW).10-11,13-17 These studies have documented a complex situation in NSW, characterised by: limited resources available for HTA; inconsistent processes for the introduction, diffusion and prioritisation of technologies; and an overall paucity of evidence for assessment. These findings are disconcerting for several reasons. First, health technologies are largely provided by public subsidy and therefore it is important that decision making be fair, transparent and accountable.18 Second, if we are not confident that new technologies are consistently and rigorously evaluated then there is a risk that resources are being used inefficiently, with consequential adverse impacts on population health.10 Third, differing processes, or lack thereof, mean that access to effective technologies may differ according to hospital or jurisdiction, raising issues of equity.

To address such issues, in 2011 the South Australian Department of Health (SA Health) implemented a governance model that incorporated a policy of systematic evaluation for high-cost medicines. Under the policy, an expert evaluation panel was convened to develop a state-wide formulary for high-cost medicines. In 2012, SA Health commissioned an external review of its governance and HTA processes in relation to non-medicine technologies. The review resulted in the establishment of state-wide advisory committees to provide recommendations on the introduction and use of high-cost[i] and/or high-risk[ii] technologies. This was intended to "support priority-setting by facilitating appropriate consideration of all health technologies in regards to safety, effectiveness, and cost-effectiveness within the wider healthcare system, prior to use within SA Health. This offers the potential to maximise beneficial health outcomes within the available budget whilst being cognisant of the other important goals of the health system".19 The committees consist of members with expertise in medical and surgical specialities, nursing, pharmacy, HTA, health economics, bioethics, health policy, and consumer representation. They are comprised of 10-20 members and meet 4-6 times per year. They are at different stages of development, with one high-cost committee being more established. These committees play an important role in supporting decision making in SA, though, like analogous national committees, they are advisory and do not make the final funding decision. The committees consider the proposed clinical pathways,[iii] systematic evidence reviews, and cost and usage data. They also consult with key clinicians and look to relevant decisions in other jurisdictions. The committees' terms of reference and processes are published and guide the preparation of detailed evidence summaries and their subsequent consideration.

Published research has yet to identify the precise criteria, evidence and standards that Australian state-level committee members actually use during their private and shared deliberations. How do committee members, individually and collectively, reason about

i Predicted expenditure is ≥ $100,000 for a Local Health Network per year or ≥ $300,000 within the SA public health system per year.

ii The technology is TGA Class III or an Active Implantable Medical Device (AIMD). Only applies to one committee

iii Clinical pathways are management plans that identify an appropriate sequence of clinical interventions, as well as time frames and expected outcomes, for a homogenous patient group. *Definition from: Clinical access and redesign unit: Queensland Health. 2013 (last update: 31 October 2013). Clinical Pathways. Retrieved 9 June, 2016, from https://www.health.qld.gov.au/caru/pathways/.20*

whether a health technology ought to be recommended for funding? Without knowledge of this, it is not possible to critically engage with how well their development of recommendations performs against stated policy objectives and how their development of recommendations compares to that of other states and of national and hospital advisory bodies.

Our study aimed to explore **the criteria, evidence and standards that committee members use to recommend funding for high-cost health technologies within South Australia.** This article reports on a qualitative study of health technology assessment at the state level in SA, with implications for comparable settings. The research focused on the committee members that are tasked with considering the evidence on safety, effectiveness and cost-effectiveness and making recommendations for the public subsidy of new health technologies.

Methods

We adopted a qualitative methodology, situated within a theoretical paradigm of pragmatism.21,22 Because the more formalised knowledge in this area is so sparse, at least in terms of published literature, a qualitative approach was deemed most appropriate. This reflects the exploratory and responsive nature of qualitative research that makes it well suited to a situation in which there are many unknowns. This choice is in line with the approach of other researchers in this area who posit that data from interviews may provide richer findings than will more quantitative methods such as surveys, which can over-simplify decisions and their context.23

Decision making within a collaborative model is influenced not only by individual beliefs and actions but also by the collective attitudes of group members and the organisation and political environment in which decisions are made. Therefore, it was important both to engage with individual decision makers (through interviews) and with the practice and context of decision making (through meeting observations). Hence data collection consisted of:

- Eight one-hour, face-to-face, semi-structured interviews with individual committee members, conducted by one researcher; and
- one observation of a meeting of each committee, conducted by two researchers.

This also allowed for data triangulation 21 in the form of comparing individual members' perspectives on decision making with the actual decision-making process in action. The research received approval from the Human Research Ethics Committee at the University of Adelaide (project nº H-2015-184). Appendix A contains the interview schedule used.
Participant selection, recruitment and data collection. Using a purposive sampling strategy,21 we invited to participate in interviews all of the members of two committees established to assess high-cost medicines and high-cost or high-risk health technologies in SA Health. The committees were also approached for observation via committee Chairs. Participants were members of one or both of the committees. The planned sample size was 10 semi-structured interviews (or until data saturation was reached) and meeting observation of two committee meetings. Previous research indicates that 10-12 interviews is sufficient to obtain 90 per cent of themes in qualitative analysis. 24 However, after a total of eight

interviews and two meeting observations, data saturation had been reached and therefore data collection was stopped: consistent views were being expressed across interviews and meeting observations were also consistent with interview data. All interviews were audio-recorded, transcribed verbatim and checked for accuracy by one researcher, while detailed electronic field notes were taken by two researchers during meeting observations. These field notes were collated and major differences in content were resolved through discussion between the two researchers. Interviewees represented 25 per cent of the total possible sample.[iv]

Coding and thematic analysis

The interview transcripts and meeting observation notes were analysed using NVivo 10 software to identify themes. Using the thematic analysis approach described by Braun and Clarke,25 data was interrogated using *a priori* codes based on the research focus. These codes were *criteria, standards,* and *evidence*. However, coding was not restricted to *a priori* topics but was also responsive to emergent themes within the data. Each source was coded fully once with coding then performed in an iterative manner, with each code and source being revisited multiple times as the researchers' understanding of the themes evolved. For example, the initial *a priori* code of *criteria* grew over time to a number of codes (including *effectiveness, budget impact* and *opportunity cost*) to reflect participants' understandings of different considerations. Appendix B contains the final list of codes used.

Findings

This paper reports on themes most pertinent to the research focus as well as the strongly emergent theme relating to committee members' perceived role in improving equity and the quality use of health technologies.[v] In presenting findings related to cost-effectiveness, it is relevant to describe two key concepts to aid in the interpretation of findings: cost-effectiveness and budget impact. Cost-effectiveness refers to how one technology compares to an alternative in terms of the differences in their costs and benefits, typically reported in the form of an incremental cost-effectiveness ratio (ICER). Budget impact simply refers to the anticipated net cost of a technology given a forecast of its rate of utilisation and any associated savings (e.g. fewer hospitalisations).

Criteria

Effectiveness

While participants described a range of criteria they apply when evaluating health technologies, their main concern was effectiveness. Participants thought of the effectiveness of health technologies broadly, along the following lines. First, participants established the nature of the potential benefit, such as an increase in mobility, or an improvement in overall survival. Secondly, they considered factors such as the availability of other treatments, the expected disease course and the significance of the benefit in terms of quality of life. The perceived importance of other factors moderated their view of the technology's potential

iv Across the 35 members of two committees there were 32 individuals, since some members sat on both committees.

v In order to preserve the anonymity of participants, identifying information has been removed from quotations and instead shown as […]. Moreover, any references to specific health technologies have been removed and replaced with the term 'health technology'. With respect to meeting observations, reference to content represents the consensus of two observers.

effectiveness. For example, a technology offering a small health benefit may be considered particularly effective if patients had limited therapeutic choices. Or a technology extending survival by only a few months, but with reduced or unchanged quality of life, may not be perceived as effective. This is illustrated in the quote below.

We are also looking at the patient groups as well and, is this, you know, is this an under-served patient group and they don't have many other options? Or you know is the kind of benefit that they're going to get really important? So is it going to help them to function to the point where they can go back to work or something like that? Or you know, or is it just going to extend their lives with low quality for another two months? So, you know, we're kind of looking at the sorts of things that were hoping to achieve by doing it.

Cost-effectiveness or budget impact

Participants were aware that costs provided a context for decision making since all technologies assessed at their level have a high unit cost or a high system-wide cost impact. However, among participants there were differences in terms of the perceived responsibility and ability of their committees to consider the cost-effectiveness and/or budget impact of a technology. It was often unclear which concept participants were referring to in discussions of cost. Some participants felt that it was not their responsibility, or that of their committee, to make recommendations based on the cost-effectiveness or budget impact of a technology. Others clearly felt that, as decision makers, they did have an obligation to consider the cost-effectiveness of technologies. For example, during a meeting of one committee the researchers observed opposing views being expressed as part of an open dialogue about whether costs should be considered in developing recommendations. At the meeting of the other committee costs were discussed in terms of consequences and numbers were given; however, it was still unclear how the costs influenced the recommendations. From interviews, it was clear that when participants consider cost-effectiveness they also exhibit reservations about their responsibility to make decisions based on it. This is illustrated in the quote below.

Each application is considered on its merits. But the issues of how the money is spent is really the decision of government rather than us. We obviously will look at cost-effectiveness but, in the end, it's the person who holds the purse strings that has to make the ultimate decision on where the money is spent. Some of these new treatments are incredibly expensive.

Participants also indicated that when cost and cost-effectiveness are considered they are usually not the dominant reasons for rejection. The primary reasons for rejection included insufficient or uncertain evidence on safety or effectiveness. This of course would likely translate to insufficient or uncertain evidence on cost-effectiveness. In deciding whether to recommend a technology, interviewees did not describe cost-effectiveness or budget impact as central criteria for rejection or recommendation. This is illustrated in the quote below.

I mean we tend not to, we do take it [cost-effectiveness] into account but it tends not to be our primary thing. And when we reject, it would usually not be just on grounds of cost. We just make recommendations, yeah, so we can never actually decide or not, we can just decide to recommend rejection. But even when we do that it wouldn't be just because we think it costs too much – it would always be on lack of evidence. That the benefit is not really great or that we think that they haven't actually defined their patient group really well, or something like that.

Additionally, participants acknowledge that the role that cost plays in a decision to recommend a technology may depend on whether a technology is seen to be *"incredibly expensive"*, in which case more attention to budget impact, cost-effectiveness or both may be given. Whether cost-effectiveness is considered can also be influenced by whether there is any economic evidence available to consider. Participants noted that, because the clinical evidence base for a technology might be poor, this translates to problems with calculating cost-effectiveness and so its consideration might be limited to instances in which that information is available. Participants also indicated that the type of consideration given to cost (cost-effectiveness or budget impact) is likely to depend on the forecast impact of the technology. For example, one participant noted that:

If it's a big thing. Like if it has a big impact on a large population they might commission a model. If not and it's only like 10 patients or something then they might just go on the financial cost.

Evidence

Evidence to apply the criterion of effectiveness

Across interviews it became clear that the technologies for which funding is sought tend to have a scant or problematic evidence base. In many cases, although committee members prefer evidence in the form of randomised controlled trials or meta-analyses, they are often operating on what some participants described as "*the edge of evidence*". Some of the intended patient populations were small and it seemed unlikely that large, well-designed trials would ever be conducted. It emerged that, although interviews need to be confident that a technology confers some patient benefit, they felt that they still needed to recommend a technology, despite uncertainty around the magnitude of benefit. The quote below illustrates a common situation for decision makers.

A lot of the time what you'll have is just a case series or two that wasn't controlled and the patients might have been selected in all kinds of biased ways. And you don't really know how they would've gone on something different or if they hadn't been treated. So you can have a look at a health technology where they reported on 10 patients and 8 of them got better. And you think, how do you know they wouldn't have got better anyway? There's no comparison, there's no sort of controlling and often no attempt to measure things really well. So it's really, really difficult and in the end you just have to go by… not your gut but I mean a sort of judgement on how good the evidence is taken all together.

Committees attempt to fill evidence gaps by drawing from local hospital data (e.g. reviewing patient records), and meeting observations indicated that individual clinical experience also informs discussion. Local data constitutes an important source of evidence, with participants placing value on being able to review the outcomes of patients treated in SA. Despite its perceived value, participants also reported difficulties in obtaining such data and in instituting monitoring post-approval, in particular. At a meeting of one committee there was discussion over the fact that they were in an area of minimal evidence, and in response there emerged a discussion asking for outcome data. Participants reported occasions where local data collected for the committee's review later fed into subsequent decisions made at the national level. Participants value local data because it can illuminate the profile of patients likely to be treated within SA and because it can help to confirm, or refute, claims of safety and/or

effectiveness. Participants also spoke about the potential to use local data to monitor the performance of technologies once funded (referred to by participants as "*outcome monitoring*"). One participant characterised outcome monitoring as being able to answer questions like the following:

Were the number of patients they expected too small or too large? Are we treating way more patients than we expected? What has been the actual cost of this?

Evidence to apply the criterion of cost-effectiveness

Interviewees consistently indicated that decision makers are more likely to have information about budget impact than cost-effectiveness. This may be in part due to the unavailability of evidence to inform cost-effectiveness analysis. Interviewees cited a lack of evidence to estimate relevant outcomes such as quality-adjusted life-years (QALYs) gained, thereby prohibiting calculation of the incremental cost per QALY gained. Participants described "*back-of-the-envelope calculations*" as useful indicators of cost-effectiveness, which speaks to challenges undertaking model-based evaluation. When considering cost-effectiveness, participants also felt that they can get a sense of whether the technology might be cost-effective. This is exemplified by the two quotes below.

We don't have specific criteria about cost-effectiveness levels, like we can hardly ever compute QALYs to be honest. […] sometimes we'll be able to do some broad ones and some things are reasonably obvious. Things that are truly cost-effective, it's usually pretty obvious usually – often what we do is we get, if we get the clinical pathway right then often it means that a health technology does become cost effective.

They don't go into more complex modelling and they tend not to introduce utilities or anything like that. So it's very basic, but it's looked at properly. Or as properly as it can be with the resources available.

Participants acknowledged that they think about opportunity cost, but that they struggle to integrate this into decisions, partly because evidence on opportunity costs may be lacking. This is exemplified by the quote below.

The thing about the opportunity costs is, it's kind of outside our remit. We do obviously think about it because any dollar that's spent on a high-cost health technology is not a dollar that's available for something else. But in reality it's not as if there's dollars sitting there and they will go here if... so it's kind of difficult to take that into account in a really structured way.

Standards

Standards to apply criteria

No clear picture emerged of what standards are used to determine whether a technology performs adequately in relation to pre-set criteria. Although participants often articulated the concept of a standard, a precise standard was not able to be identified from interviews. Standards tended to be vague. The quote below illustrates this:

So we make decisions, I mean unless it's an outrageous, obvious waste of money or there's no health gain. I mean that's the other waste of money, if there's no years of life gained or no reduction in symptoms or disability then why would you pay for that? So we make decisions

at that level but primarily our focus is on improving health outcomes, lessening injury and disability, and improving somebody's life. So it's about safety and quality.

Researcher: *Yeah, ok, so it's about looking at something and saying 'well, that does have some benefit'?*

Yes, but the benefit has to be enough. Because it's taxpayers' money.

The above quote implies that a standard (though vague) is being applied: *"the benefit has to be enough"*.

Overall it was not clear from interview or observation data, what the precise standards were or how committees deem them to have been met. For example, no ICER thresholds were discussed.

The role of decision makers in considering equity and the quality use of health technologies

Participants viewed promoting consistency in clinical practice and the evidence-based, quality use of health technologies as an important part of their committee's role. Decision makers exhibited concern regarding the way the technology under review should be used and how the care leading up to and including the use of that technology should be delivered. Through review of clinical pathways, decision makers identify areas in which current practice may be inconsistent and make recommendations regarding components of care that can and should be standardised. If a technology is recommended, it may be recommended with criteria for access that assist in reducing potentially inappropriate use. Participants viewed this process as an essential part of their work and thus felt that they contribute to building *"as strong an evidence base as possible for medical practice"*. Participants identified inequities in access to health technologies as a problem affecting both SA and other Australian jurisdictions. They described this as an important rationale for implementing a state-wide approach to considering health technologies. Common views are illustrated in the quotes below.

I think some people think we're just an instrument of the government trying to reduce costs. And that may be true to some extent but, for me, the equity issue is important and I think we can improve quality of care with this process.

For these patients not being able to access their health technology because that doctor only practices at that hospital and not their hospital – then that's not fair.

Further to this, meeting observation revealed active consideration of these issues which is represented in the field notes below:

The revised pathway was proposed by the applicant. The committee is debating whether it is adequate/sufficiently comprehensive. […] One person comments [that we may wish to avoid having multiple first-line options in SA] Discussion focuses on the opportunity for standardisation.

Discussion

The discussion follows the logic of the research focus with consideration given to criteria, evidence and then standards. Research from the institutional and local health network level in

NSW10-11,13 and around the world26 is consistent in finding that safety and effectiveness are considered the most important criteria in health technology funding decision making.

The findings of our study reinforce this body of literature, with effectiveness being the criterion of central concern for participants. Participants' nuanced description of effectiveness indicates that when considering effectiveness, decision makers are really considering the value of a technology in terms of its potential to improve an individual patient's well-being. This includes the technology's safety profile. It follows that committee members at the state level in SA are unlikely to recommend a technology with little or no evidence of patient benefit. Committee members acknowledge that cost and cost-effectiveness are not considered as main reasons for rejection, though they may pay attention to budget impact when they deem costs associated with the technology to be extremely high.

This finding indicates that committee members intend to safeguard patients from harmful and ineffective technologies whilst recommending access to safe and effective technologies. Furthermore, because the committees provide state-wide oversight with respect to the introduction and use of high-cost technologies, patients in different parts of SA should have equal access to recommended technologies as a result. Participants often referred to this as an important reason for having such processes in place. This is an important achievement of the committees, since one of the criticisms of Australia's fragmented approach to healthcare funding and governance (with federal and state bodies having distinct but overlapping responsibilities) is the potential for inequity across jurisdictions.13 Other literature from sub-national levels within Australia has found that decision makers are concerned about inconsistent decision making across local areas and about resultant inequities in access to health technologies between hospitals.13 Participants in our study felt empowered to consider inconsistencies in access and practice and to make recommendations that would bring about positive change. Participants see state-wide decision making as a vehicle for increasing consistency in care regarding both access to a particular technology and the care leading up to it.

There are good reasons why decision makers at many levels are tasked with considering cost-effectiveness. Because resources are scarce, decisions regarding the funding of health technologies should arguably reflect the aim of maximising health benefits within available resources. Therefore, a key consideration should be the opportunity cost associated with that decision. By evaluating both the costs and effects of alternative health technologies, economic evaluation provides information on how to minimise the opportunity cost. Considering opportunity cost speaks to the following question: after funding a technology, is more health likely to be gained than lost across the population? This question is difficult to answer but, in principle, fundamental to the aim of maximising population health.

Within this research we observed some differences among participants in terms of whether they think their specific committee ought to consider cost-effectiveness and budget impact. Some participants viewed cost-effectiveness as outside their remit (as individuals or as a committee) or as beyond their capacity to assess. Information on budget impact was more commonly available for consideration than cost-effectiveness analyses. Participants describe a situation where cost-effectiveness in terms of an ICER is often unknown. If information on cost-effectiveness is not available, or not considered, recommendations are mainly based on the effectiveness of the technology under consideration as opposed to being based on whether

funding it would serve to maximise population health (i.e. cost-effectiveness evidence). As per national processes, in SA the question of whether a technology can be afforded (budget impact) is likely to be considered by decision makers at or nearer to the executive level. The committees we studied are not responsible for identifying a source of funding for the coverage of new technologies.

We found that the committees we studied do not systematically use cost-effectiveness evidence, for reasons we discuss below. It may be that some committee members take their task to be primarily to identify the clinical risks and benefits associated with a technology and that the issue of cost-effectiveness is, like the issue of budget impact, the responsibility of other decision makers who more keenly face cost pressures. However, it would be reasonable to infer that, once a technology is recommended by the committees studied, decision makers would need to present a strong argument as to why the technology does not then warrant funding. This was something alluded to within our meeting observation (see Finding 2.2).[vi]

There is a large volume of research from around the world that has found economic evaluations to be under-utilised by local decision makers, including in Canada, the United States, England, and Australia.13,15,23,26-30 This volume of research is also consistent with the finding of studies describing decision-making processes at the local level in the Australian state of NSW.11,17 Sub-national bodies in Australia differ from national bodies such as PBAC and MSAC in that national bodies have come to *require* economic evidence as part of submissions and thus will not recommend a technology without economic evidence. This difference is not confined to Australia. Eddama and Coast 28 found that decision making at the level of primary care trust in the United Kingdom has not followed the lead of the National Institute for Health and Care Excellence (NICE) in always utilising economic evaluation. In line with their terms of reference, state-level committees in SA apply safety and effectiveness as fundamental criteria. However, cost-effectiveness does not, in practice, appear to function as a fundamental criterion, in that economic evidence is not *always* required for a technology to be assessed and potentially recommended.[vii] Additionally, not all members are convinced that, if available, cost-effectiveness evidence should inform their recommendations.

Perhaps the greatest barrier to the consideration of cost-effectiveness at the sub-national level related to a lack of evidence and limited resources to commission new economic evaluations. Our participants highlighted a paucity of economic evidence as an important barrier to the consideration of cost-effectiveness.[viii] Cost-effectiveness analysis is limited by the quality of the available data on both effectiveness and costs, 31 and decision makers may not have the

vi In meeting observation (see Finding 2.2), discussion reflected that technologies with positive recommendations do tend to get funded; however, it was not possible to determine the exact number of positive recommendations that resulted in funding as public summary documents are not available for every technology reviewed.

vii We use the terms 'economic evidence' and 'economic evaluation' as technical terms to refer to formal types of analysis such as cost-effectiveness analysis, cost-utility analysis and cost-consequence analysis. We do not use them to refer to a consideration of budget impact.

viii Interviewees also tended to see applications as needing to pass a clinical hurdle in that if they are not safe or effective then it is not necessary to consider costs as they would be rejected on clinical grounds before they got to the economic considerations. In some instances where the clinical evidence is scant or unavailable it may be that cost-effectiveness is not discussed owing to failure at this first hurdle (see Findings 2.2 and 2.5).

expertise or resources available to conduct cost-effectiveness analyses when none are already available in the literature.

A number of further reasons have been advanced as to why economic evaluation is under-utilised in sub-national decision making. Research in NSW has suggested that clinicians and the public lack an understanding of economic evaluation and its role in decision-making, that decision makers are uncertain about the feasibility of undertaking it at the local level, and that decision makers often see problems with applying existing economic evidence to the local context.10 Related to the first of these, another potential reason relates to committee membership. Sub-national committees tend to feature mainly clinical specialists, who may have a preference for effectiveness over cost-effectiveness.23 Clinicians have dual obligations to act as good stewards of scarce resources (with impacts on population health) as well as to pursue the best interests of their patients.32 In the setting of a clinical encounter, the patient should arguably settle for no less than the clinician doing everything possible to benefit the patient in accordance with the patient's needs and wishes.33 However, outside of the clinical encounter, clinicians may be expected to take on the role of a public decision maker before whom the interests of different individuals and groups may come into conflict.33 This shift in mindset may be difficult, in that it asks clinicians to exit the clinical encounter and to put to one side medicine's traditional ethic of acting in the best interests of one's patient.

Having discussed criteria, we now discuss evidence. Recommendation of a health technology can be interpreted as indirect evidence of its usefulness and can undermine the case for undertaking further research.34,35 Griffin et al. 34 argue that new technologies are being adopted and reimbursed increasingly earlier in the life cycle of technologies which reduces incentives to undertake new research. This can ultimately undermine the evidence base for current and future practice and impact on health gains obtained from current and future decisions. Our findings indicate that state-level committee members in SA apply a high level of scrutiny to clinical evidence and are well aware of various shortcomings of different trial designs. This study also identified that state-level decision makers place importance on and utilise local outcome data in decisions. By using local data, they may be able to supplement a poor evidence base, identify potential benefits or disadvantages associated with the use of a technology in the local context, and tailor recommendations to this context, where the case-mix or comparators may differ from those of other jurisdictions. This was not a finding reported by other studies of local decision making in Australia, suggesting that SA may be a leader in this regard.

However, we did not identify specific minimum clinical evidence requirements for funding consideration by state-wide committees in SA. This is partly driven by the recognition that it may be unethical or impractical to collect strong evidence for every intervention. Decision makers at the state-level have little assurance that future evidence will emerge if they reject a technology and therefore need to balance the potential benefits of approval to current patients against the benefits to future patients offered by the more robust evidence base that can result from withholding approval.35 A lack of evidence, or poor-quality evidence, is a problem that also affects MSAC and PBAC,8,36 but these committees are able to contract independent HTA agencies to conduct assessments of technologies or provide critiques of sponsor-prepared applications that can identify and quantify uncertainty. Moreover, because of the

ability of MSAC and PBAC to affect total utilisation they may have more influence on industry research agendas.

Finally, we could not identify precise and explicit standards that the committees we studied apply to determine whether a technology is good enough with respect to a specific criterion. Furthermore, it does not appear that there is an articulated policy directive regarding a threshold of clinical effectiveness or cost-effectiveness that can be applied by members. However, not all technologies are recommended; some applications meet thresholds of acceptability while others do not. One way to interpret this, suggested by our observations and interviews, is that technologies need to jump a minimum hurdle of comparative effectiveness, i.e. they must provide a benefit to patients that exceeds that offered by existing alternative treatments. Therefore, some standard is being applied with respect to effectiveness; however, the exact nature of the standard is difficult to identify as an observer and perhaps difficult to articulate as a participant. Once this hurdle is jumped it is not clear whether other criteria, such as cost-effectiveness, also need to be met and what standards might be applied when considering those criteria.[ix] There may be good reasons to retain flexibility in decision making (e.g. to accommodate unique contextual factors or complex and unique interactions among factors). However, an absence of explicit standards does raise important questions of: whether the implicit standards being applied are consistent between technologies; whether they reflect the goal of maximising or optimising population health; and what other moral imperatives they reflect and how closely aligned those are to community priorities. The implications of using implicit standards that decision makers may, or may not, be conscious of is that it becomes difficult to explain decisions and challenging to engage critically with the process.

With that said, the processes of the committees we studied do conform in important ways to the model of procedural justice advanced by Daniels.38[x] The committees we studied provide public summaries explaining their decisions. The criteria these cite are incontrovertibly relevant (e.g. sound evidence of effectiveness). And decisions are, in principle, revisable, with early efforts being made to collect local data on utilisation, costs and patient outcomes following decisions.

The committees represent a proactive approach to the complex environment of health care decision making by making use of available data to make recommendations. Consistent with other jurisdictions, they face important challenges with respect to considering cost-effectiveness, due to a paucity of clinical and economic evidence and other factors. By

ix Campbell and Knox37 describe a model of decision making that occurs in the UK in a setting of limited available evidence. This model describes a context in which, where there is limited clinical evidence, promise and plausibility have a role in making a choice under scarcity. They describe the idea of cost-consequences, which involves considering the cost of adopting a new technology in place of a clearly defined existing service. This takes into consideration direct and indirect costs or savings associated with the technology. Taken together, this allows decision makers to consider whether introduction would increase costs, with the expectation of better outcomes, or perhaps save costs, with the expectation of equal or better outcomes compared to an existing service. The authors note that this requires balancing promise, risk and cost. This model may in some way help to understand the approach taken by the committees studied that do not routinely have cost-effectiveness analyses available but certainly consider in some way the promise, risk and costs of technologies.

x Daniels argues that a choice under scarcity should be: (1) transparent to the public; (2) made on the basis of reasons that all parties agree are relevant; (3) revisable in the light of new evidence and arguments; and (4) guaranteed to be all these things through some mechanism. These conditions enable decision makers to 'account' for how 'reasonable' their choice is under scarcity.

attempting to mobilise local data and undertake outcome monitoring, the committees may be able to draw on a more complete and rich data set; however, these activities are at present limited due to resourcing challenges. Further investment in HTA at the state level may help to overcome these barriers in the future.

Limitations

This study was conducted within SA and involved participants from only two of what are a number of committees that make and consider recommendations and decisions on health technologies. It is not clear whether the findings are transferable either to other states within Australia or to decision making at different levels within SA (e.g. hospital levels). Additionally, this study provides limited insight into the decisions made below state-level committees that may have a greater impact on population health than those of the state-level committees, simply by virtue of volume.

With respect to data collection it is important to note only a subset of members of the committees were interviewed and observation was only of one meeting of each committee. Our presence was abnormal for the committee, members understood that we were there for research purposes, and a number of the members were known to the researchers. This may have influenced the meeting in ways that would be hard to identify.

In considering these limitations, it should also be noted that despite our relatively small sample, in our judgement, data saturation with respect to criteria, evidence and standards was reached and observations from two separate meetings provided important data triangulation. Similarly, participants in this study were key informants, i.e. those whose experience in this area is substantial and should speak to the issues interrogated.

Another important contextual consideration is that the researchers have experience with HTA and therefore bring to the interpretations of findings pre-conceived ideas about HTA and its application to policy at the state level. This background will have shaped the understanding and interpretation of findings. However, the use of two researchers for observations reduces the risk of one researcher overly biasing interpretation. The primary researcher also used semantic coding, which focuses on the explicit content of the data.

Conclusions

In Australia, few studies have been conducted on the development of funding recommendations at the state level. Within SA, we found that committee members tasked with developing funding recommendations at the state-wide level carefully assess evidence on safety and effectiveness. The committee members' reasoning is grounded in the desire to improve outcomes for patients, and decisions may safeguard public patients from harmful and ineffective technologies while providing access to safe and effective technologies. Committee members also described success in contributing to consistency in care and equitable access to technologies. However, decision making is challenging and frequently technologies have a scant or problematic evidence base. At the state level, cost-effectiveness does not function as a fundamental criterion insofar as formal economic evaluations are not uniformly required for a technology to be evaluated for public subsidy. This stands in

contrast to the approach taken at the national level. Broadly speaking, the lack of use of cost-effectiveness evidence in a systematic way may have adverse effects on the efficient use of resources, with consequential impacts on population health. Moreover, there is some variation in participants' views or understandings of the responsibility of their committees to consider cost-effectiveness. As per national advisory committees, the standards used are implicit, therefore it was not clear what standards are applied to determine when a technology ought to be recommended. The challenge of incorporating economic analyses, in terms of their availability or perceived utility, appears pronounced at sub-national levels. Future research could seek to understand how outcome monitoring could be best resourced and implemented to overcome barriers with respect to limited available information and to retrospectively evaluate decisions.

Disclosures, compliance with ethical guidelines and competing interests

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Appendix A - Supplementary data, interview questions

Can you tell me about your role and how it relates to decisions made about funding health technologies?

Can you describe how you make decisions on funding health technologies?

Who is involved in the process?

What information do you need to make a decision?

Are there any explicit criteria used in decision making?

If yes, can you describe these criteria and how they are used and do you know where they came from?

Are there any minimum information requirements for a decision to be made?

Once you have that information how do you make a decision? Are there particular considerations that are of more or less importance to decision making?

Are economic evaluations used to make decisions? How does the cost of a technology factor into the decision making process?

How much do you think the process you describe is unique to your role and/or how much do you think it is common to other decision makers in South Australia?

Do you think the committee is important?

What would trigger a technology to be referred to the committee?

Can you describe the main challenges to health technology funding you think exist for decision makers in South Australia?

Appendix B - Supplementary data, list of codes from analysis in NVivo

Table 1 Supplementary data regarding analyses: list of codes

Codes (parent codes containing sub-codes are bolded, indents denote the level of coding)	Number of sources*	Number of references
Criteria	**10**	**175**
Safety, effectiveness and cost-effectiveness	10	138
Costs or cost-effectiveness	10	94
cost-effectiveness	7	24
Opportunity cost	7	22
trade-offs	2	3
Budget impact	8	18
CE Threshold and ICER	5	8
Patient costs	5	8
Effectiveness related content	8	31
Patient benefit	8	28
Clinical pathways	5	7
Patient opinion or role	3	5
Window of therapeutic benefit	1	1
Safety	6	13
Decision making criteria (general)	9	37
Evidence	**10**	**79**
Expertise	8	21
Uncertainty	4	7
Values, shared perceptions, judgements bias	**9**	**73**
Decision makers comfort with process	8	17
Shared values	8	15
Group decision making	4	15
Rigour	6	14
Subjective or projected judgement	2	5
Bad behaviour or practices	1	5
Inherent value	1	1
Vested interests or bias	1	1
Contextual issues, may be criteria	**10**	**70**
Defining patient populations	3	13
Political influence	4	12
Comparators	5	10
Quality of life	6	9
standard care	4	8
Palliative or compassionate procedure	1	4
Utilisation of the technology	4	4
Underserved patient groups or lack of therapeutic options	2	3
Age distribution of the population	2	3
Vulnerable groups	2	2
best practice	1	1
Mechanism of action	1	1
Roles	**10**	**67**

Roles, perceptions and identities of Clinicians	9	59
Clinician attitudes	9	35
Role of doctors	7	21
Clinical champions	2	3
Role of decision-makers	3	8
National committees (PBAC or MSAC)	**9**	**40**
PBAC	6	30
MSAC	3	7
HealthPACT	3	3
Local committees or groups or considerations	**9**	**32**
SA characteristics	6	18
SA Health	3	8
State formulary	3	3
Drug and Therapeutics committee	2	2
Local new technologies committee	1	1
Standards	**7**	**29**
Health outcomes	3	5
Industry	9	29
Process	6	29
Comparing drugs and devices	7	26
Thresholds or triggers	7	22
Other jurisdictions	**8**	**16**
Other states	4	5
Monitoring	**6**	**15**
Outcome data	7	19
Consistency	6	15
Decisions	**7**	**15**
Rejection	5	12
Disinvestment or later repeal of decision	3	3
Entry of technology to market	**6**	**13**
Uncontrolled entry or use	2	2
Governance	**6**	**13**
Quality use of medicines	6	9
Equity of access	6	11
Confusion	5	10
Barriers to evaluation	5	7
Interview	7	7
Challenges or negatives associated with committees	2	5
Training and upskilling	2	4
centralisation	1	2
Public health	1	2
long term evidence	1	1
acceptability of cost	1	1

*total possible = 10