

**Trial of Program Budgeting and Marginal
Analysis (PBMA) to Assist Cancer Control
Planning in Australia**

[PBMA Series No 5: Summary Report]

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ACKNOWLEDGMENTS

The Health Economics Unit of the CHPE is supported by Monash University.

The Program Evaluation Unit of the CHPE is supported by The University of Melbourne.

Both units obtain supplementary funding through national competitive grants and contract research.

The research described in this paper is made possible through the support of these bodies.

AUTHOR ACKNOWLEDGMENTS

A project as demanding as this PBMA trial inevitably owes thanks to a range of people for their time and commitment, particularly when it is undertaken in a tight time frame. The project team would particularly like to acknowledge the invaluable contribution and support of members of the Working Party. The knowledge, guidance and intellectual contribution of the Working Party members was an important ingredient to the success of the trial. Special thanks must go to Professor Bruce Armstrong (Chair) and Professor Alan Coates (Deputy Chair) for their tireless contributions throughout all stages of the trial.

We would also like to acknowledge the gracious and helpful secretariat services provided by the Commonwealth Department of Health and Aged Care, particularly the assistance of Mr Stephen Nerlich and Ms Elizabeth Butkus. Our thanks go to Stephen in particular, for facilitating the survey of the Cancer Strategy Group members reported in Chapter 6; for assisting in the acquisition of HIC data; and in liaising with various areas of the Commonwealth Department.

Individual members of the team would also like to acknowledge with gratitude the advice and assistance of experts and practitioners in the field with the preparation of their briefing papers. In particular, our thanks go to Dr Heather Mitchell, Medical Director of the Victorian Cervical Cytology Registry, for assistance with the Cervical Cancer Screening paper; to Ms Doreen Ackerman and Ms Amanda Hordern of the Victorian Anti-Cancer Council (ACCV) for assistance with the Breast Care Nurse paper; to Dr James St. John, Director of Gastroenterology at the Royal Melbourne Hospital, for assistance with the Colorectal Cancer Screening Paper; to Dr David Hill, Chair of the National Expert Advisory Committee on Tobacco (NEACT) for advice on the appropriate tobacco control option to evaluate; and to Dr Helen Dixon of the ACCV for assistance with the effectiveness data on the Victorian "Fruit n Veg" campaign.

The project was jointly funded by the Commonwealth Department of Health and Aged Care (National Health Priorities and Quality Branch, Health Industry and Investment Division (HIID), together with the Victorian Department of Human Services (Public Health and Development Division) on behalf of the Cancer Strategies Group (CSG) of the National Health Priorities Committee.

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Acronyms

AIHW	:	Australian Institute of Health and Welfare
CBT	:	Cognitive Behavioural therapy
CSG	:	Cancer Strategy Group
DALY	:	Disability Adjusted Life Year
DCIS	:	Disease Costs and Impact Study
DHAC	:	Commonwealth Department of Health and Aged Care
EUROQoL (5D)	:	That form of the EuroQoL that has five dimensions against which quality of life is assessed
EUROQoL	:	European Quality of Life Assessment Instrument
GBD	:	Global Burden of Disease Study
HPV	:	Human Papilloma Virus
ICD-9	:	International Classification of Disease – Revision 9
MEEM	:	Macro Economic Evaluation Model
NCCI	:	National Cancer Control Initiative
NHMRC	:	National Health and Medical Research Council
NHPAs	:	National Health Priority Areas
PBMA	:	Program Budgeting and Marginal Analysis
PV	:	Present Value
WHO	:	World Health Organisation
YLD	:	Years Lived with a Disability
YLL	:	Years of Life Lost

1 Abstract

1.1 Background

In mid-1999, the Cancer Strategy Group (CSG) of the National Health Priorities Committee, resolved to trial the use of the Program Budgeting and Marginal Analysis (PBMA) approach. The trial was to address whether PBMA is an appropriate technique to include in CSG's strategic planning process, as well as assist with what specific options might be included in the next National Cancer Strategy.

1.2 Methods

Advocates of the PBMA approach believe it offers a practical way of applying the economic principles behind the achievement of "allocative" and "technical" efficiency. It is argued that PBMA allows the concept of "benefit" to be clearly related to program and/or organisational objectives and for best available evidence to be utilised in an open and systematic planning process. It is clear from the literature that substantially different evaluation approaches are possible within the overarching framework of PBMA. The key characteristics of the PBMA approach adopted in this trial were:

- a focus on the marginal analysis component of PBMA, combined with a clear rationale for selection of the options for change;
- an evidence-based approach with a small research team bringing together the best available information;
- the choice of the Disability Adjusted Life Year (DALY) as the measure of health gain;
- the recognition that "benefit" is broader than just health gain;
- adoption of a two-stage approach to the assessment of benefit involving both "technical" aspects (i.e. cost per DALY recovered; level of evidence) and "judgement" aspects (i.e. equity; size of the problem; acceptability to stakeholders; and feasibility of implementation); and
- an economic protocol specifically developed for a priority setting context.

1.3 Results

Eight options for change, involving both increments (additional expenditure) and decrements (reduced expenditure) were evaluated and ranked according to their cost per DALY results. Information was produced on their gross and net cost; DALYs recovered; and anticipated cost

effectiveness ratios. Such information should facilitate informed decision-making within a budget context.

All increment options were considered suitable for inclusion within the National Cancer Strategy, with the lowest ranked option (colorectal cancer screening) having a net cost per DALY of \$10,300 – a cost effectiveness ratio better than the current national breast and cervical cancer screening programs. Of the two decrement options evaluated, one (extending the interval for cervical cancer screening from two to three years) was considered suitable for adoption. Application of the second-stage filters did not alter the ranking of the interventions, but increased the Working Party's confidence in the validity of the results. In the case of the cervical cancer screening decrement, the "acceptability" filter raised issues that would warrant careful consideration before adoption.

There are good reasons to support the credentials of options evaluated in the PBMA trial (both in terms of their selection for evaluation and their evaluation results) but this should not be overplayed. There may well be other potentially cost effective interventions available that were not evaluated as part of the PBMA trial. Some of these may well be raised on a priori grounds for inclusion in the National Cancer Strategy alongside the PBMA evaluated projects.

The results from the PBMA trial were compared with the results from the consensus-based approach to priority setting undertaken by National Cancer Control Initiative (NCCI) in 1997 and with a survey of CSG members conducted prior to the PBMA trial. The comparisons confirmed the importance of:

- clarity as to the concept of benefit;
- clarity as to the "decision rules" in ranking proposals and clarity as to when such rules are modified by judgement reflecting broader criteria (eg. equity, acceptability, feasibility); and
- quality information on the cost, outcomes, effectiveness and efficiency of interventions.

The attention in the PBMA process to these issues led to a clearer rationale for the results achieved (that could be subject to later scientific review and revision) and greater consensus between the participants. The range in the scores given and/or size of the standard deviations in the CSG survey, for example, suggested a lack of shared understanding on evaluation criteria and project performance. In this context the use of mean scores from such surveys could be quite misleading if they were used to imply a consensus view.

1.4 Discussion

The merit of the PBMA approach was discussed on several occasions in the various meetings of the Working Party. The clear view that emerged from these discussions is that while there were aspects of the PBMA methods and process that could be criticised and/or improved upon, the

PBMA approach trialed represented a “*quantum leap forward in the quality of information available for decision-making*” (Professor Mark Elwood). Both the information produced, together with the process by which this information was assembled, were viewed in a very positive light. More specifically, the use of an evidence-based approach facilitated by a suitably qualified research team assembling information was strongly supported. The type and quality of information, the method of its collection and presentation, and the clarity as to its intended use were all viewed as important improvements over the NCCI priority setting process of 1997.

At a more detailed level, the PBMA trial was also attempting to test issues such as:

- the ability of PBMA to deal with quite divergent options in the disease pathway from prevention through to palliation;
- its ability to measure and weight benefits involving multiple dimensions and different levels of evidence;
- its integration of both “technical” and “consensus” approaches to priority setting;
- its ability to break down priority setting into manageable tasks; and
- its likely acceptability to stakeholders.

While time prohibited all the intended options for change from being assessed, the trial nonetheless provided a sound basis for assessing whether the evidence-based approach to PBMA is feasible and whether it constitutes a sensible approach to priority setting. The options covered health promotion, illness prevention, diagnosis and care components of the disease pathway, as well as both mortality and morbidity dimensions of health gain. Members of the Working Party concluded that the PBMA process performed well in relation to these issues and that an evidence-based PBMA approach has the clear potential to be an important component of the planning process for cancer control in Australia. The exact nature of that involvement requires further careful thought. The more important issues to be considered here include:

- the organisation and coordination of any ongoing PBMA work program, including its integration with CSG and NCCI activities;
- the provision of adequate resources and a feasible timetable to sustain any ongoing PBMA program;
- the strengths and weaknesses of the DALY as the prime measure of health gain;
- the research and data implications of an ongoing PBMA program of work (eg. development of equity weighting; DALY disaggregated into target groups; maintain the Australian Institute of Health and Welfare, Disease Costs and Impact Study (DCIS) data base; develop the efficacy/effectiveness data base);
- whether the program structure and program budget aspects of PBMA should be activated; and
- integration with any broader consultation process.

2 Background

The Cancer Strategies Group (CSG) oversees the development of a National Cancer Strategy in Australia. A key element of CSG's approach to its task is a systematic decision-making process for priority setting and strategy development.

In 1997 the National Cancer Control Initiative (NCCI) was launched. It was based on the conviction that it should be possible to get a better return for expenditure on cancer than was currently being obtained and that it was timely to introduce new evidence-based cancer control measures. The NCCI undertook an extensive consultation process involving organisations in Australia with an interest in cancer control and developed a set of consensus-based priorities for cancer control that would have an effect within 5 years. The findings were published in the "*Cancer Control Towards 2002*" report (NCCI, 1998). Subsequent discussion of the strengths and weaknesses of the NCCI report included the issue of whether an economic approach could be utilised as part of the decision-making process for developing cancer control priorities. In particular, the discussion focussed on whether all the participants assessed "benefit" attributable to the various interventions in a consistent way and whether the anticipated benefits could be related to resource use in a clearer and more overt fashion.

In mid-1999, the CSG resolved to trial the use of the Program Budgeting and Marginal Analysis (PBMA) approach as part of its review of the priorities determined in the "*Cancer Control Towards 2002*" report. The trial was to address whether PBMA is an appropriate technique to include in the planning process, as well as assist with what specific options might be included in the next National Cancer Strategy. CSG also agreed that the top 20 projects identified in the NCCI "*Cancer Control Towards 2000*" report would form the starting point for the PBMA options assessment. A Working Party chaired by Professor Bruce Armstrong was constituted to both manage and participate in the PBMA pilot. A Project Team led by A/Professor Rob Carter and Ms Christine Stone conducted the PBMA and associated economic appraisals.

3 The PBMA Approach

PBMA offers a practical approach to applying the economic principles behind the achievement of "allocative" and "technical" efficiency. It examines how benefits or health gains to individuals and populations may be maximised for a given budget covering a wide range of services and interventions. It allows the concept of "benefit" to be clearly related to program and/or organisational objectives and for best available evidence to be utilised in an open and systematic planning process. The basic steps involved in conducting a PBMA study are:

- agree on the research question and essential features of the PBMA protocol;
- decide on a program structure that matches the research question;
- describe the program in activity and expenditure terms (i.e. the "program budget");

identify the options for change (both increments and decrements);
undertake marginal analysis of the options;

- 5.1 establish objectives of the organisation and/or program and develop the approach to measurement of benefits;
- 5.2 assess benefit of options with the instrument and/or approach developed in 5.1;
- 5.3 assess net costs of the options; and
- 5.4 estimate the PBMA cost-effectiveness ratios using 5.2 and 5.3 (and undertake sensitivity analysis)

assess and discuss the PBMA results, including comparison with any existing appraisals and/or broader dimensions of benefit included as second stage filters; and

consider recommendations/design implementation strategy (if appropriate to research question).

In this pilot study it was agreed that the research question would focus on steps 5 and 6, with some attention given to step 4.

4 The Options Evaluated

The options for change in this PBMA pilot were identified through a three-step process. First, in accordance with the pilot specifications, the starting point was the 'top 20' priority actions from the NCCI report "*Cancer Control Towards 2002*" (NCCI, 1998). Second, a National Cancer Strategy Development Workshop (Commonwealth Department of Health and Aged Care [DHAC], 1999a) provided the opportunity to re-visit these strategy areas and to add options to the NCCI 'top 20'. This step allowed developments since completion of the NCCI report to be taken into account, together with options that may have been excluded by the NCCI process. Third, the Working Party assessed the resulting list of 21 action areas (involving over 40 individual interventions and/or activities) and classified them into one of five groups using a number of criteria, viz:

- that a clear and concrete intervention could be specified;
- that there was sufficient evidence to make an assessment of efficacy/effectiveness possible;
- that both increments (i.e. options that involve additional expenditure) and decrements (i.e. options that involve reduced expenditure) be included;
- that options from across the complete disease pathway (i.e. from prevention to palliation) be included;

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- that options be included that test the assessment of both mortality and/or morbidity impacts on health status; and
 - that the a priori perceived importance of options be taken into account, as reflected in the NCCI rankings (*NCCI, 1998*), a survey conducted of CSG members (*DHAC, 1999b*) and the National Cancer Strategy Development Workshop (*DHAC, 1999a*).

The five groups were:

- 1 *Options for change* – defined as interventions where sufficient evidence exists to indicate that strategies involving additional expenditure would be associated with significant health gain and strategies involving decreased expenditure would be associated with little or no reduction in health gain.
- 2 *Possible options for change* – defined as interventions where some evidence exists to indicate that strategies involving additional expenditure would be associated with health gain and strategies involving decreased expenditure would be associated with little or no reduction in health gain. These options may need more work to specify to a level of precision where they can be evaluated.
- 3 *Monitor developments/liaison* – defined as interventions that are currently being worked on and/or implemented in another context and where it is too early or inappropriate to perform an economic evaluation at present.
- 4 *Research strategies* - defined as possible interventions that need more research before they can be evaluated, that is evidence does not yet exist to sustain their efficacy/effectiveness credentials and a clear intervention cannot be specified.
- 5 *Motherhood strategies* – defined as those ideas for action that were considered to have merit but were too broad and abstract to evaluate (and for which specific research work was not developed).

The intention was to include in the PBMA analysis all those strategies classified as “*Options for change*,” together with some of the “*Possible options for change*.” The full list of all potential options considered by the Working Party, allocated to one of the five categories listed above, is at Appendix 2 in the full report. An abbreviated listing of the strategy areas intended for inclusion in

the pilot is set out below¹. The option numbers given in brackets are identical to those used in the report of the National Cancer Strategy Development Workshop (*DHAC, 1999a*) and in the survey of CSG members (*DHAC, 1999b*) at Appendix 8 of the full report.

Options for Change

- 1 Reducing Smoking Prevalence - (option 2.2.)*
- 2 Reduce the risk of skin cancer - (option 4.1.)*
- 3 Improve skills in diagnosing skin cancer - (option 5.1.)
- 4 Improve efficiency of cervical screening - (option 6.2.)*
- 5 Improve detection of colorectal cancer - (options 7.1 & 7.2.)*
- 6 Rationalise prostate specific antigen testing - (option 10.1.)
- 7 Develop guidelines in areas of need - (option 12.2.)
- 8 Improve palliative care: guidelines for pain management - (new option in strategy 14.)
- 9 Define, implement and monitor psychosocial care – (new options in strategy 15.)*

Possible Options for Change

- 10 Increase consumption of fruit and vegetables through health promotion– (option 3.4.)*
- 11 Organise education and resources for those with familial cancers – (option 18.1 & 18.2.)
- 12 Meet urgent national needs in data collection – (option 20.2.)

Briefing papers to guide the Working Party in its PBMA assessment were prepared on six strategy areas (asterisked above), involving eight options for change. The briefing papers are quite detailed assessments that summarise the available evidence on each option². Section 6 of this report is based on these briefing papers, which are prepared to the following standard layout:

¹ Evaluation of two strategies in the “options for change” list (i.e. palliative care and guidelines in areas of need) could not be commenced due to the short time scale available. Similarly, two of the three “possible options for change” could not be included (i.e. familial cancers and national data collection needs). The decision on which options to omit reflected the expertise of the research team; the anticipated time required to research the options; and the ranking of the options by the NCCI and the CSG. Two further strategies were commenced (skin cancer diagnosis skills & PSA testing) but were put on hold either due to staff availability constraints and/or data availability constraints. Data to support the evaluation of the skin cancer diagnosis intervention, for example, will become available later this year from trials being conducted in South Australia. Pending the availability of this data, it was sensible for the evaluation of this intervention to be put on hold.

² Due to the length of these briefing papers, they are listed in Appendix 3 of the full report and are not included as part of either the summary or full reports. All the briefing papers will be published separately, however, as part of the Centre for Health Program Evaluation’s (CHPE) Research Paper Series and will be made available for downloading from the CHPE web site.

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- the intervention is first clearly defined;
 - the potential health benefit is assessed in terms of Disability Adjusted Life Years (DALYs)³, with epidemiological assumptions clearly specified;
 - the net health service costs are estimated, with economic assumptions specified;
 - the cost effectiveness ratios (average and marginal, where appropriate) are presented;
 - the second stage filters (i.e. equity; public health significance; level of evidence; and acceptability to stakeholders; and feasibility of implementation) are considered in terms of their impact on the priorities established by the cost per DALY results.

5 The Measurement of Benefit

Clarity as to the concept of benefit is an important aspect of the PBMA approach. After reviewing the goals and objectives of the National Cancer Strategy, the Working Party adopted seven broad criteria, viz:

- 1 the size of the problem (i.e. where can the biggest difference be made?);
- 2 the efficacy/effectiveness of the intervention (i.e. what is the quality of the evidence that the intervention works and what health status improvement can be anticipated?);
- 3 the capacity of the intervention to reduce inequity in health status and the health care system (a multi-factorial issue involving population groups, process and outcomes);
- 4 the efficiency of the cancer control intervention (i.e. is the option value for money as reflected by the economic analysis?);
- 5 the cost of the cancer control intervention (i.e. is the intervention affordable?);
- 6 the acceptance by stakeholders, particularly the general community; and
- 7 the likelihood of successful implementation (i.e. availability of relevant expertise and/or infrastructure; timing considerations; or other feasibility issues).

It was agreed by the Working Party that these seven criteria would be utilised in a two-stage approach to ranking the options in the marginal analysis. In the first stage options would be ranked by those criteria directly related to determining the resources consumed or released by the option, together with the size and distribution of the anticipated health gain. In the second stage the ranking of options would include the more pragmatic acceptability/feasibility issues. The

³ A DALY is the sum of the years of life lost (YLL) and the years lived with a disability (YLD). This measure can be applied in each of the options identified by the Working Party. The model being used is based on the Burden of Disease approach used by the Australian (Mathers et al, 1999) and the Victorian (Vos & Begg, 1999a,b) studies. Refer Appendix 3 of the full report for further detail.

first stage is characterised by aspects that lend themselves to “logical” decision-rules, drawn essentially from the disciplines of health economics and epidemiology. The second stage incorporates aspects where it is very difficult to develop decision-rules and decisions rest heavily on judgement and due process.

5.1 First Stage Health Benefit

The principle dimension of health benefit was the estimated *size of health gain* associated with each option. The size of health gain is a quantitative measure, the calculation of which was evidence-based using a combination of the scientific literature and expert opinion. This dimension was calculated from the total DALYs attributed to the disease and the efficacy/effectiveness of the option in reducing that DALY burden. Appendix 3 of the full report summarises the procedures involved and provides a worked example using colorectal cancer screening.

The Working Party’s intention was for the DALY score attributed to each intervention to be weighted for equity based on the health status implications for four target groups, viz. socio-economic status; Aboriginal and Torres Strait Islander peoples; rurality; and ethnicity. While some of the issues associated with using equity weights have been examined, applied case studies could not be finalised in the time available. It is premature at this stage to offer any definitive judgement as to the practicality and acceptability of weighting cost effectiveness results for equity. It is clear, however, that if equity is to be included in an *evidence-based approach*, then there are some important implications for future research directions. In this regard it should be noted that there are some simpler and more tractable approaches available for the inclusion of equity in the PBMA toolkit (using decision theory), but they would not satisfy the stipulation that the approach be evidence-based.

5.2 Second Stage Health Benefit

The second stage involved the more pragmatic issues that may impact on the implementation of an option⁴; together with those factors that influence the degree of confidence⁵ that can be placed in the PBMA cost effectiveness ratio. These issues are defined and discussed in the report. The approach to the “level of evidence” filter warrants preview here, however, given the prominence given to evidence in the PBMA approach adopted.

The Working Party was aware that the National Health and Medical Research Council’s (NHMRC) thinking about assessing evidence has moved on since publication of “*A guide to the development, implementation and evaluation of clinical practice guidelines*” (NHMRC 1999). In particular, there is increasing recognition that there *is a single framework* within which evidence

⁴ Such as: equity (which could not be included in Stage One in the time available); size of the problem; acceptability to stakeholders and feasibility

⁵ Such as: level of evidence; comparative cost effectiveness results from conventional economic evaluations.

on clinical, public health and social science interventions can be assessed. While the nature of the evidence for different kinds of health interventions will inevitably vary, and the evidence for public health and social science interventions will often be weaker than that for clinical interventions, the logic used to assess the evidence is the same for all of them.

In considering this development, the Working Party noted the approach taken by the “*best buys*” team in New South Wales.⁶ The decision was taken to follow this approach, modified to reflect the classification used by the International Agency for Research in Cancer. The combined approach is illustrated in Table A.

Table A Classifying the Strength of the Evidence

Strength category	Strength of the evidence
<p>“Sufficient evidence of effectiveness (or ineffectiveness)”: Effectiveness (or ineffectiveness) is demonstrated by sufficient evidence from well-designed research.</p>	<ul style="list-style-type: none"> • The effect is unlikely to be due to chance (eg., P is <<0.05) and • The effect is unlikely to be due to bias (eg., evidence from: <ul style="list-style-type: none"> Ⓔ a level I study design; Ⓔ several good quality level II studies; or Ⓔ several high quality level III-1 or III-2 studies from which effects of bias and confounding can be reasonably excluded on the basis of the design and analysis)
<p>“Limited evidence of effectiveness (or ineffectiveness)”: Effectiveness (or ineffectiveness) is demonstrated by limited evidence from studies of varying quality</p>	<ul style="list-style-type: none"> • The effect is probably not due to chance (eg., P is <0.05) but • Bias, while not certainly an explanation for the effect, cannot be excluded as a possible explanation (eg., evidence from: <ul style="list-style-type: none"> Ⓔ one level II study of uncertain or indifferent quality; Ⓔ evidence from one level III-1 or III-2 study of high quality; Ⓔ evidence from several level III-1 or III-2 studies of insufficiently high quality to rule out bias as a possible explanation; or Ⓔ evidence from a sizeable number of level III-3 studies which are of good quality and consistent in suggesting an effect).
<p>“Inconclusive evidence of effectiveness (or ineffectiveness)”: Inadequate evidence due to insufficient or inadequate quality research.</p>	<ul style="list-style-type: none"> • No position could be reached on the presence or absence of an effect of the intervention (eg., no evidence from level I or level II studies and level III studies are available, but they are few and of poor quality, or only level IV studies are available.)

⁶ Which made a distinction between the robustness of the research methodology (using the NHMRC Quality of Evidence Rating Scale (NHMRC, 1996)) and the more important “level of evidence” conclusion. The NSW approach (NSW Health Department, 1999) involved the following categories:
 “Evidence of effectiveness”: Effectiveness is demonstrated by clear evidence from well-designed research.
 “Inconclusive Evidence”: Effectiveness cannot be demonstrated due to insufficient or inadequate quality research.
 “Evidence of ineffectiveness”: Ineffectiveness is demonstrated by clear evidence from well-designed research

6 Results of the PBMA Analysis

6.1 Methods

The results of the economic evaluations presented in Section 5 of the full report can be brought together to provide a “league table” in which the interventions are ranked in order of their economic merit. Economists urge caution (quite rightly) in simplistic use of league tables. For the information in league tables to be of use to decision-makers, they need to be confident that the methodology of the source studies (contained in the table) is sound and that it is relatively homogeneous across the various studies. Secondly, most cost effectiveness or cost utility league tables include studies from a range of settings and economic data may not be transferable from one setting to another. Thirdly, there are issues associated with the use of decision rules in practice, particularly in the presence of a budget constraint. These pitfalls have been avoided in the present study because the evaluations have been undertaken specifically within a priority-setting context, rather than being an ad hoc collection of studies assembled from the literature. More specifically, the key features of the economic protocol are:

- 1 The rationale for the selection of interventions in the league table is clearly explained and consistently applied;
- 2 Evaluation methods have been standardised and clearly specified;
- 3 Setting and context is common to all interventions (i.e. to be part of a National Cancer Strategy);
- 4 Australian data has been used for demography, health system costs, disease incidence, risk factors; and
- 5 The costs per DALY ratios are placed within a broader decision-making context provided by the PBMA approach.

6.2 Selection and Description of Competing Alternatives

An important question in critically assessing economic evaluations is whether any important alternatives were omitted. This relates both to the correct specification of the “project case” options (i.e. the options for change), as well as to the “base case” comparators (i.e. the status quo). In the context of an economic evaluation addressing a single topic or problem, there is usually a reasonably limited set of possibilities. In the context of a study addressing priority setting across a disease group as broad as cancer, there is a very broad range of possibilities. This then raises an important theoretical and policy issue, the process by which the options for change were selected. While the process of selection is clearly explained in Section 4 of this report, it needs to be acknowledged that the list of “project cases” was by no means comprehensive. In part this was due to the constraints applying to the trial in terms of available

resources and time; in part it is due to the constraints applying to any priority setting exercise in terms of available resources and information bases.

The net effect of this discussion is the importance of recognising that the projects evaluated through the PBMA trial should not be given undue prominence. There are good reasons to support their credentials (both in terms of their selection for evaluation and their evaluation results) but this should not be over-played. There may well be other potentially cost effective interventions available which were not evaluated as part of the PBMA trial and some of these may well be raised on a priori grounds for inclusion in the National Cancer Strategy alongside the PBMA evaluated projects.

6.3 Cost per DALY Results

Table B provides a summary of the cost per DALY results. The interventions are ranked on the basis of the economic evaluation results – either by their level of “dominance” or the net cost per DALY ratio. Dominance is a term that is used in economic evaluation to describe the situation where an intervention is estimated to yield both cost savings and health status gains. Note that three interventions, all addressing important risk factors (i.e. sun exposure; smoking; and inadequate diet), are estimated to be “dominant.” Tobacco control is ranked above primary skin cancer prevention as both the anticipated cost offset is higher (i.e. \$39.0 M versus \$37.4 M) and the anticipated DALYs recovered is higher (i.e. 10,599 DALYs to 9,965). When interventions are not dominant (i.e. the more normal circumstance where net expenditure is involved to secure health gains) they are ranked on the basis of the net cost per DALY ratio. The net cost per DALY estimates provided include the point estimate (i.e. the result from the primary economic analysis) together with the upper and lower bound estimates from the sensitivity analysis using the @RISK software.

Note that for some interventions (eg. colorectal cancer screening) a specific design option has been selected (eg. biennial screening of those aged 55-69). More detailed results showing the marginal analyses of different design options (particularly for colorectal cancer screening) and sensitivity analyses are provided in Section 5 of the full report. The marginal results for colorectal cancer screening suggest that the inclusion of the 70 – 74 age group is cost effective, and whilst costing an additional 12.4 million, improves the efficiency of the program. The Working Party was of the view that the core colorectal cancer screening program should be seen as biennial screening of those aged 55 – 74. For this reason the results for this design variant are also shown in Table B.

Dominant programs have very strong economic credentials for funding. There is nonetheless a practical financial issue for government that needs to be flagged. “Opportunity cost savings” are estimates of resources devoted to the treatment of preventable diseases (or de facto screening programs) that could be available for other purposes. Conversion of opportunity cost savings into

financial savings involves a number of practical and theoretical considerations⁷ and should not be taken for granted. It is for this reason that both gross and net cost estimates have been reported in Table B. If the interventions had been ranked on the gross cost per DALY ratios (i.e. excluding the cost offsets), then the ranking would be slightly different. Primary prevention of skin cancer and tobacco control would swap their positions as first and second; as would the fruit & vegetables media campaign and the breast care nurse intervention as third and fourth.

The changes considered to Australia's national cervical cancer screening program are likely to be contentious. While every attempt has been made to use representative and up-to-date information, the reliance on Victorian data for a range of parameters⁸ should be noted and the sensitivity analysis examined to assess the impact of parameter variations.

Before turning to the implications of Table B, it is also important to note that both increments and decrements⁹ have been included. If a decrement both saves resources and causes no loss in health status, then it is also "dominant" as for the increments. Unfortunately, this is an unlikely occurrence, unless existing programs are providing no benefit or causing harm. The more likely result is that there will be a small increase in the DALY burden as the opportunity cost of securing the resource savings. If the results of decrements with this outcome are expressed as a ratio to make them comparable with those increments where health gains involve a positive net cost, they need to be carefully interpreted. The easiest way to understand the ratios is to express them in the negative i.e. they are the cost per DALY of maintaining current practice and not accepting the proposed change. Thus the cost per DALY of not increasing the screening interval from two to three years for cervical cancer screening is \$516,864, involving an opportunity cost of approximately \$50 million per year.

One of the key implications of the table relates to what conclusions might be drawn in relation to resource allocation. If, for example, a budget of \$50 million were available, where would it be spent? Is it possible to fund such a budget from the decrements? Would the benefits from investing the \$50 million in the increments outweigh the loss in benefits in the decrements?

The possibility of introducing a national colorectal cancer screening program is being considered at present and provides a useful case study to address these questions. The introduction of a colorectal cancer screening program providing biennial screens for men and women aged 55-69

⁷ The practical issues include workforce re-structuring, management policies, political acceptability, professional interests and public reaction. The theoretical issues relate to the cost characteristics of the production function, involving factors such as the mix of 'variable' costs and 'fixed' costs, together with 'lumpiness' in the expansion/contraction of capital equipment and assets.

⁸ As the main report details in section 5.3.3, the parameters based on Victorian data include number of women participating in the program; the number of women who had a further assessment & treatment; the unit costs of smears read in public labs; and recruitment, coordination, registry, and training costs. The survival assumptions rely heavily on South Australian and NSW data.

⁹ The decrements involving PSA testing & skin cancer diagnosis are not yet available.

is estimated to cost approximately \$53.3 million per annum (excluding offsets). Such a program is estimated to reduce the disease burden from this cancer by 3,187 DALYs each year. This intervention, however, ranks last amongst those evaluated in terms of its net cost per DALY ratio. If that same \$50 odd million were used to finance the five interventions that rank above it in our league table, then the DALY burden in the various diseases involved would be reduced by 31,993 DALYs. Such analysis suggests that the interventions ranked above the colorectal cancer screening option have stronger economic claims for funding from our hypothetical budget.

Table B Ranking of the Interventions on the Basis of the Degree of Dominance and Net Cost per DALY Results

Interventions	Cost (Savings) \$ Millions	DALYs Recovered (Lost)	Cost per DALY (Point estimate) \$	Cost per DALY (Lower bound) \$	Cost per DALY (Upper bound) \$
INCREMENTS:					
1 st : Tobacco Control: National Tobacco Campaign					
Gross Costs (no offsets)	8.95	10,599	844	544	1,180
Net Costs (or net saving)	(39.07)		Dominant	Dominant	Dominant
2 nd : Primary Prevention of Skin Cancer (SunSmart on National Basis)					
Gross Costs (no offsets)	2.53	9,965	254	238	505
Net Costs (or net saving)	(37.4)		Dominant	Dominant	Dominant
3 rd : Fruit & Vegetables Media Campaign					
Gross Costs (no offsets)	2.46	3,626	677	513	16,392
Net Costs (or net saving)	(12.15)		Dominant	Dominant	Dominant
4 th : Psychosocial Care: Breast Care Nurses					
Gross Costs (no offsets)	4.85	5,186	935	455	1,745
Net Costs (or net saving)	Offsets not estimated				
5 th : Psychosocial Care: Psychologists for Cancer Treatment Centres					
Gross Costs (no offsets)	25.7	4,849	5,292	1,612	5,453
Net Costs (or net saving)	Offsets not estimated				
6 th : National Colorectal Cancer Screening Program (Biennial: Ages 55-69)					
Gross Costs (no offsets)	53.3	3,187	16,718	12,500	44,800
Net Costs (or net saving)	38.1		11,958	10,300	39,700
(Biennial: Ages 55-74)					
Gross Costs (no offsets)	65.6	4,260	15,399		
Net Costs (or net saving)	43.8		10,300		
DECREMENTS:					
1 st : Rationalise the National Cervical Cancer (CrCx) Screening Program: Change Screening Interval from 2 to 3 years (Net Saving)	(50.6)	(98)	516,864	156,172	955,407
2 nd : Rationalise the National CrCx Screening Program: Increase age of commencement from 18 to 25 (Net Saving)	(23.7)	(30)	790,996	624,242	959,259

If we consider the decrements as the source of our budget, then the suggested interval change in the cervical cancer screening program would provide the funds at an opportunity cost of increasing the DALY burden from cervical cancer by 98 DALYs per year. Any such conscious increase in the DALY burden deriving from policy decisions carries with it important equity and ethical issues. On the other hand, the equity and ethical implications of not making these changes, if their implementation was contingent on savings being found from existing expenditure, would also need to be considered. Clearly the DALY benefit from either application

mentioned above (i.e. a 3,187 DALY reduction from introducing colorectal cancer screening and a 31,993 DALY reduction from the interventions ranked 1st to 5th) considerably outweighs the DALY increase.

6.4 Impact of the Second Stage Filters (including Equity)

Table C provides an overview of the Working Party's discussion of the second stage filters. If the filters were treated as dichotomous constraints (i.e. "pass" or "fail") then none of the interventions would be ruled out by them. There are, nonetheless, some important issues raised that should not be ignored. These include the design of the interventions (eg. colorectal cancer screening); the need to strengthen the evidence base (eg. psychosocial care by breast care nurses; fruit & vegetables); and the way in which proposals might be implemented (eg. cervical cancer screening).

7 Comparison of PBMA Ranking with the 1997 NCCI Results

7.1 Background

The NCCI undertook an extensive consultation process involving organisations in Australia with an interest in cancer control and developed a set of consensus-based priorities for cancer control that would have an effect within 5 years. The process adopted by the NCCI involved:

- the generation of a list of 36 topic areas in cancer control;
- the development of proposals for up to 10 actions by expert working parties assigned to each of the 36 areas;
- a winnowing process by which 19 key stakeholders, including the NCCI Management Committee and Priority Steering Committee, reduced the number of proposed actions from 276 to 147;

Table C Summary of Cost per Daly Results and Impact of Second Stage Filters

Options (Increments & Decrements)	First Stage Filter: Cost per DALY	Equity Implications	2 nd Stage Filter: Levels of Evidence	2 nd Stage Filter: Size of the Health Problem	Acceptability & Feasibility
<p>1. Colorectal Cancer Screening (increment)</p> <p>Decision points:</p>	<p>Biennial 55-69 age group: \$11,958 per DALY recovered (net cost est). Biennial 55-74 age group: \$10,282 per DALY recovered (net cost est). Annual 55-69 age group: \$16,039 per DALY recovered (net cost est). Addition to biennial program of: 70 to 74 age group: \$5,277 per DALY (net cost estimate). 75+ age group: \$5,956 per DALY (net cost estimate). 50-54 age group: \$23,111 per DALY (net cost estimate). 45-49 age group: \$32,616 per DALY (net cost estimate).</p> <p>Important design features to be tested, with significant cost implications (ie age; interval; attendance; positivity rate; equity issues). Introducing another national screening program requires very careful consideration due to health system inertia.</p>	<p>There are existing inequities in the incidence & mortality from CRC for low SES, rural/remote and Aboriginal/Torres Strait Islanders.</p> <p>Introduction of a National screening program is likely to increase existing inequities (due to likely utilisation patterns) unless specific action is taken to address equity issues in design & implementation of program.</p> <p>Consideration of this filter raises important issues for program design.</p>	<p>“Sufficient well-designed research.”</p> <p>Major international trials (4 of 6) reported a reduction in mortality of 12-21% for biennial screening. Only one RCT available for annual screening.</p> <p>Consideration of this filter increases the weight to be given to a successful intervention.</p>	<p>High on health burden and health system costs. CRC is second most common cancer affecting both men & women. Medium on potential reduction in the disease burden (ie 11% fall in DALYs with biennial screening; 17% with annual). High on potential costs of screening program and potential cost offsets.</p> <p>Consideration of this filter increases the weight to be given to a successful intervention.</p>	<p>Financial cost varies hugely depending on design option (ie \$38 million to over \$180 million.)</p> <p>Acceptability issues for participants include availability of counselling and adequate information on cost, benefit & risk.</p> <p>Quality assurance (including positivity rate) will need to be established</p> <p>Physicians still need to be convinced of efficacy of CRC screening.</p> <p>Major feasibility issue is health system inertia – will be hard to wind-up and harder to wind-down if that becomes necessary.</p> <p>Consideration of this filter suggests caution and need for careful planning.</p>

Table C Summary of Cost per Daly Results and Impact of Second Stage Filters (Cont'd)

Options (Increments & Decrements)	First Stage Filter: Cost per DALY	Equity Implications	2 nd Stage Filter: Levels of Evidence	2 nd Stage Filter: Size of the Health Problem	Acceptability & Feasibility
<p>2. Cervical Cancer Screening (decrements)</p> <p>Decision points</p>	<p>Option One: 2 yr screening vis-à-vis 3 yr screening: \$516,864 per DALY in staying with current policy.</p> <p>Releases \$50.6 million per annum.</p> <p>Option Two: 25 yr age of commencement: \$790,996 per DALY in staying with current policy Releases \$23.7 million per annum.</p> <p>Strong case on efficiency & efficacy grounds to consider change in interval. External validity of data assumptions needs to be kept in mind.</p> <p>Acceptability remains a major obstacle (as it will for most decrements). HPV screening may provide opportunity for change. Proposed use of released funds key issue.</p>	<p>There is higher health burden from cervical cancer in lower SES groups, in remote areas, in some migrant groups and in indigenous women.</p> <p>Proposed changes unlikely to increase inequities associated with screening program. Rather, quite the reverse is true, particularly if some of the savings are used to address inequities in the program.</p> <p>Consideration of this filter is at worst neutral and at best supportive of option.</p>	<p>Option One: “Sufficient well-designed research”.</p> <p>Option Two: Knowledge of the impact of varying age of commencement is inconclusive with a rating of: “Limited evidence of effectiveness”.</p> <p>Reliance on Victorian data needs to be kept in mind re external validity.</p> <p>Consideration of this filter supports the change in interval, but raises reservations about the change in age of commencement.</p>	<p>For Australia as a whole, cervical cancer has a low health burden. The higher incidence and mortality rates in rural/ remote localities and for indigenous women remain a concern however.</p> <p>Potential adverse health effects of proposed changes are minor, particularly when assessed against potential gains elsewhere.</p> <p>This filter is potentially very supportive of this option.</p>	<p>Any proposed change to the National program is likely to face strong opposition from medical practitioners. Use of savings key issue re acceptability.</p> <p>Reaction from the community will depend on how well the changes are explained and communicated, together with use of resources saved.</p> <p>Future action in relation to HPV may provide vehicle for change.</p> <p>This filter is the major concern for this option.</p>

Table C Summary of Cost per Daly Results and Impact of Second Stage Filters (Cont'd)

Options (Increments & Decrements)	First Stage Filter: Cost per DALY	Equity Implications	2 nd Stage Filter: Levels of Evidence	2 nd Stage Filter: Size of the Health Problem	Acceptability & Feasibility
<p>3 Primary Prevention of Skin Cancer: Sunsmart on National basis (increment)</p> <p>Decision points:</p>	<p>\$254 per DALY recovered (excluding cost offsets) Dominant if cost offsets included (ie saves resources and improved health status).</p> <p>Few complications with this proposal, but requires Government cooperation and agreement on funding sources.</p>	<p>Incidence varies directly in relation to intensity of and exposure to UVR. Only target group where equity issue may arise is rural/remote areas. Greatest financial impact will be on private individuals conforming with Sunsmart policy guidelines. Equity filter is largely neutral for this intervention.</p>	<p>Good evidence exists that educational campaigns can impact on behaviour. A complex chain of events is nonetheless assumed, between behaviour and disease reduction. "Limited evidence of effectiveness" supports this intervention. Sufficient evidence exists to support intervention.</p>	<p>Skin cancer (particularly NMSC) is very high on health burden and health system cost. Potential impact of intervention is significant. Consideration of this filter greatly enhances merit of intervention.</p>	<p>This intervention is comparatively low cost, requiring little development. It involves little system inertia, being easy to wind-up or contract. High community acceptance anticipated. This filter also supports intervention.</p>
<p>4. Improve efficiency of diagnosing skin cancer</p>	<p>Pending the availability of trial data from SA, it was sensible for the evaluation of this intervention to be put on hold.</p>				
<p>5. Psychosocial care: Breast Care Nurses (increment)</p> <p>Decision points:</p>	<p>67 full-time Breast Care Nurses: \$935 per DALY recovered (indicative but robust result).</p> <p>Whether evidence base is strong enough to support widespread implementation or whether more cautious approach required.</p>	<p>The BCN intervention is unlikely to worsen any existing inequalities and provides potential to address special needs groups. Access in rural/remote areas may be a problem This filter does not pose a problem.</p>	<p>"Limited evidence of effectiveness" exists from studies of varying quality. This filter suggests caution as to the next steps to pursue this intervention.</p>	<p>Breast cancer imposes a major health burden and significant health system costs. Potential impact on breast cancer morbidity is substantial for low cost intervention (\$5 m). This filter supports option.</p>	<p>Likely to be widely acceptable if efficacy sustained. Feasibility in rural/remote areas needs to be explored. Supply of BCNs in states other than Vic/NSW may need to be addressed through training. No significant issues posed by this filter.</p>

Table C Summary of Cost per Daly Results and Impact of Second Stage Filters (Cont'd)

Options (Increments & Decrements)	First Stage Filter: Cost per DALY	Equity Implications	2 nd Stage Filter: Levels of Evidence	2 nd Stage Filter: Size of the Health Problem	Acceptability & Feasibility
<p>6. Psychosocial care: psychologist in cancer centres (increment)</p> <p>Decision points:</p>	<p>Introduce psychologists (approx 290) for cancer patients: \$5,292 per DALY recovered.</p> <p>Funding arrangements for the proposal. Assistance from psychologists is less acceptable to equity target groups.</p>	<p>This intervention is unlikely to worsen any existing inequalities and provides potential to address special needs groups.</p> <p>Access in rural/remote areas may be a problem</p> <p>This filter does not pose a problem.</p>	<p>A number of randomised trials (as well as three recent meta-analyses) support intervention. Efficacy of proposal supported by "sufficient well-designed research."</p> <p>This filter does not pose a problem, for appropriate interventions.</p>	<p>As intervention addresses morbidity associated with a range of cancers, it is high on health burden and health system costs.</p> <p>Has potential to significantly reduce cancer morbidity.</p> <p>This filter supports intervention.</p>	<p>Acceptability to medical community and consumers is uncertain.</p> <p>Cost of intervention may be an issue to Government.</p> <p>This filter poses minor concern for the intervention.</p>
<p>7. Rationalise prostate specific antigen testing (decrement)</p>	<p>PBMA economic appraisal not yet available. Data to support the evaluation will become available from trials being conducted in South Australia.</p>				
<p>8. Tobacco control: National Tobacco Campaign</p> <p>Decision points:</p>	<p>\$844 per DALY recovered (excluding cost offsets).</p> <p>Dominant if cost offsets included (ie saves resources and improves health status).</p> <p>Second stage filters confirm strong credentials of this intervention.</p>	<p>The results of surveys of the NTC indicated that the positive effects applied to males and females; to older and younger smokers; and to all levels of educational attainment.</p> <p>Consideration of this filter does not suggest a problem.</p>	<p>Causal links between smoking and disease is now firmly established by "well-designed research."</p> <p>Effectiveness of the NTC in modifying smoking behaviour is documented by evidence from behavioural pre and post campaign survey research.</p> <p>This filter does not pose a problem.</p>	<p>Smoking is commonly acknowledged as the most important source of preventable disease and health care expenditure.</p> <p>Consideration of this filter strongly supports intervention.</p>	<p>Continuation and development of the NTC is likely to be widely acceptable and quite feasible.</p> <p>This filter reinforces the desirability of this intervention.</p>

Table C Summary of Cost per DALY Results and Impact of Second Stage Filters (Cont'd)

Options (Increments & Decrements)	First Stage Filter: Cost per DALY	Equity Implications	2 nd Stage Filter: Levels of Evidence	2 nd Stage Filter: Size of the Health Problem	Acceptability & Feasibility
<p>9. Encourage consumption of fruit and vegetables</p> <p>Decision points:</p>	<p>\$677 per DALY recovered (excluding offsets).</p> <p>Dominant if cost offsets included (ie saves resources and improves health status).</p> <p>Second stage filters confirm credentials of intervention. Clearer costing data would be important, together with strengthening of the evidence base.</p>	<p>Evaluation of the Victorian campaign suggests existing inequalities in the consumption of fruit & vegetables would be reduced. Impact on four target groups unknown, but not anticipated to be a concern.</p> <p>Consideration of this filter does not suggest a problem.</p>	<p>Causal links between inadequate consumption of fruit & vegetables and disease established by "sufficient" evidence.</p> <p>Effectiveness of intervention in modifying behaviour supported by "limited evidence."</p> <p>Sufficient evidence exists to sustain intervention.</p>	<p>The diseases causally related to inadequate consumption of fruit & vegetables are major causes of premature mortality and morbidity</p> <p>This filter reinforces the desirability of this intervention.</p>	<p>Development of a National "Fruit 'n' Veg" campaign is likely to be widely acceptable and quite feasible.</p> <p>This filter reinforces the desirability of this intervention.</p>

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- sending a questionnaire on the 147 proposed actions to every organisation with interests bearing on cancer control in Australia (667 questionnaires to stakeholders);
 - conducting workshops in each State and Territory, attended by 242 expert participants, to discuss 30 proposed actions given priority in the questionnaire responses; and
 - selection by the NCCI Management Committee of 13 proposed actions for priority implementation from a final set of 21 short-listed proposals.

7.2 Results

Table D presents the ranking of the interventions assessed in the PBMA trial set alongside the ranking of these interventions in the NCCI consensus-based approach. The NCCI columns report the place of each intervention in terms of the questionnaire results for the 147 proposed actions, the top 21 short-listed proposals, together with an indication of whether or not they made the list of 13 interventions recommended for priority implementation. The key findings arising from this comparison are:

- The exact match between the top two interventions in the PBMA trial (i.e. tobacco control and primary skin cancer prevention) and the NCCI top two interventions. The merit of some interventions clearly stand out under a range of priority setting approaches. The PBMA approach provides a clearer rationale for the ranking.
- The omission of skin cancer primary prevention from the final 13 for priority implementation seems questionable with the benefit of hindsight. The decision may have reflected the time frame (i.e. interventions must have an impact within 5 years); the view that activities were already taking place at the State/Territory level - albeit poorly funded – and/or the absence of information at that time clearly articulating the strength of its economic credentials.
- The omission from the whole NCCI process of options to rationalise the national cervical cancer screening program, even though the economic arguments and overseas precedents for these changes had been known for some time (i.e. since the national evaluation in 1990). While the PBMA analysis confirms the earlier economic results, the acceptability of any changes to the program to key stakeholders will remain a stumbling block, as it will for any of the decrements. The potential use to which any resources released through rationalisation are put may be an important consideration here, together with changes to the program related to Human Papilloma Virus (HPV) research.
- The potential merit of encouraging the consumption of fruit and vegetables is very different between the two priority setting exercises. The ranking of third in the PBMA study is much stronger than its 79th out of 147 in the NCCI survey. Undoubtedly the NCCI result reflects the lack of rigorous data on this intervention, which remains a problem today.

While the economic results from the PBMA trial must be regarded as indicative only – given the data assumptions that had to be made – the results are nonetheless impressive. Any intervention that holds promise of a net resource saving while delivering substantial health gains must be taken very seriously.

Table D Comparison of PBMA Ranking with the 1997 NCCI Results

Cancer Intervention	PBMA Ranking	NCCI: Included in the 13 priority actions	NCCI: Ranking in the short-listed 21	NCCI: Ranking in the 147 (and score out of 5)
INCREMENTS:				
Tobacco Control	1 st	Yes	1 / 21	2 / 147 (3.25)
Skin Cancer Prevention	2 nd	No	2 / 21	5 / 147 (3.13)
Encourage Fruit & Veg. Consumption (Note: cost per DALY result indicative only)	3 rd	No	Not in top 21	79 / 147 (2.51)
Psychosocial Care	4 th & 5 th	Yes	14 / 21	75 / 147 (2.53)
Colorectal Cancer Screening	6 th	Yes	7 / 21	12 / 147 (3.02)
DECREMENTS:				
Rationalise Cervical Cancer Screening	Saving of \$50.6 m & small DALY increase	No. (Not raised in the NCCI exercise)	No. (Not raised in the NCCI exercise)	No. (Not raised in the NCCI exercise)
Rationalise Prostate-Specific Antigen Testing	Results not yet available	Yes	8 / 21	15 / 147 (2.99)
Rationalise and Improve Skin Cancer Diagnosis Skills	Results not yet available	Yes	9 / 21	8 / 147 (3.08)

- The psychosocial care strategy performed creditably in both rankings, but the rationale is clearer in the PBMA trial (where it reflects the cost per DALY result). For the NCCI ranking, the strategy moved from 75th out of the 147 to 14th in the top 21 and then into the final 13 without any clear rationale being apparent. In some ways this is not surprising, because the provision of psychosocial care remains a somewhat controversial area, particularly for those not familiar with the available research. This was also demonstrated, for example, in the survey of CSG members (see Section 8) with a wide range in the scores given for this option.

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- The colorectal cancer screening proposal performs more strongly in the earlier NCCI survey than the PBMA trial, which is interesting given its epidemiological and economic credentials have strengthened in the last few years. While this proposal comes in last of the increments considered in the PBMA trial, its cost utility result (i.e. \$12,000 approx per DALY) is stronger than both the current cervical and breast cancer screening programs.

8 Comparison of PBMA Ranking with the Survey of the Cancer Strategies Group

8.1 Background

Early on in the discussions of a possible PBMA trial, it was decided to survey the CSG for its ranking of the interventions being considered for inclusion in the PBMA. The decision was taken as both a precautionary measure – in case the PBMA trial could not be completed in the short time available – and to measure the contribution of the PBMA process to the deliberations of the CSG. A copy of the survey instrument is at Appendix 8 in the full report. Members were asked to score 46 possible cancer control actions against the following eight criteria¹⁰:

- **size of the problem** each action seeks to address;
- **effectiveness** of the action (quality of the evidence basis, size of impact on the problem, capacity of the strategy to satisfy consumers);
- **cost** of the cancer control action;
- **efficiency** of the cancer control action;
- capacity to **reduce inequity**;
- **acceptance** by the community;
- likelihood of successful **implementation** (because of availability of relevant expertise, budget implications, political issues); and
- **overall importance**.

For each intervention, CSG members were asked to score the eight criteria with a number between 1 (lowest score) and 5 (highest score). As with the NCCI survey instrument, members were left to weight the various criteria themselves in arriving at the “o

¹⁰ These eight criteria were subsequently discussed and developed by the Working Party into the benefit measurement approach used in the PBMA trial (refer Section 5).

8.2 Results

Table E provides the summary results of the CSG survey for those interventions assessed in the PBMA trial, set alongside their corresponding PBMA ranking. The CSG columns report the results for all eight dimensions combined, for the “overall importance” dimension only, and for the “efficiency” and “equity” dimensions combined. The key findings from the CSG survey and a comparison with the PBMA results are:

- With one exception (colorectal cancer screening) there is reasonable consistency between the CSG survey ranking based on “all eight dimensions combined” and the “overall importance” dimension. The “efficiency and equity combined” score gives a quite different ranking for a number of interventions, particularly tobacco control, colorectal cancer screening, rationalising cervical cancer screening and psychosocial care. Similarly, analysis of individual dimensions confirms that, as expected, interventions rank differently depending on the dimension selected. The CSG results confirm the concern that led to the PBMA trial – i.e. that clarity as to the concept of benefit is important. Attaching different weights to the various dimensions scored would yield quite different rankings.
- The ranking of tobacco control and skin cancer prevention as 1st and 2nd in the PBMA trial and the NCCI is confirmed by the CSG results. A greater focus on skin cancer primary prevention seems widely accepted.
- The fruit and vegetables intervention is again ranked quite differently in the CSG survey to the PBMA trial, even on the “efficiency and equity combined” criterion. The lack of specific economic evaluation data in both the NCCI and the CSG surveys would have contributed to this result. The high standard deviation result for this intervention suggests a divergence of views, possibly due to a perceived lack of reliable data.
- Psychosocial care is ranked similarly in both the PBMA and CSG studies. The CSG gave this intervention a higher ranking than the NCCI respondents. As with the NCCI results, however, the width of the standard deviation suggests a divergence of views on the merit of this proposal.
- Colorectal cancer screening was judged to be important by the CSG (ranked 2nd on “overall importance” dimension) but ranked much lower when all dimensions were combined. Interestingly, it was given a low score on the “efficiency” dimension, even though the PBMA evaluation suggests its efficiency credentials are better than existing screening programs.

Table E Comparison of the PBMA Ranking with the CSG Survey Results

Cancer Intervention	PBMA Ranking	CSG Survey: Score for all 8 dimensions combined	CSG Survey: Score for "Overall Importance"	CSG Survey: Score for "Efficiency" & "Equity" combined
INCREMENTS:				
Tobacco Control	1 st	1 st (6 / 46)	1 st	4 th
Skin Cancer Prevention	2 nd	2 nd (8 / 46)	2 nd (tie)	2 nd
Encourage Fruit & Veg. Consumption (Note: cost per DALY result indicative only)	3 rd	8 th (26 / 46)	6 th (tie)	7 th
Psychosocial Care	4 th & 5 th	4 th (17 / 46)	6 th (tie)	3 rd
Colorectal Cancer Screening	6 th	7 th (25 / 46)	2 nd (tie)	6 th
DECREMENTS:				
Rationalise Cervical Cancer Screening	Saving of \$50.6 m & small DALY increase	5 th (19 / 46)	5 th	1 st
Rationalise Prostate-Specific Antigen Testing	Results not yet available	3 rd (14 / 46)	2 nd (tie)	5 th
Rationalise and Improve Skin Cancer Diagnosis Skills	Results not yet available	6 th (23 / 46)	8 th	8 th

8.3 Conclusions

The comparison of the PBMA results with the CSG survey (and with the earlier NCCI results) confirms the importance of:

- clarity as to the concept of benefit;
- clarity as to the "decision rules" in ranking proposals and when such rules are modified by judgement reflecting broader criteria; and
- the provision of quality information on the cost, outcomes and efficiency of interventions.

The attention in the PBMA process to these issues led to a clearer rationale for the results achieved (that could be subject to later scientific review and revision) and greater consensus between the participants. The range in the scores given and/or size of the standard deviations in the CSG survey suggests a lack of common understanding or consensus on the results. In this context the use of mean scores from such surveys could be quite misleading if they were used to imply a consensus view.

9 Evidence-Based PBMA as an Approach to Priority Setting

9.1 Background

The CSG is seeking the development of a transparent systematic decision-making process for priority setting and strategy development. Such a process would not only have a clear rationale for the decisions taken (with due regard to the best available information), but would also make values explicit and be accountable to stakeholders.

The priority setting process conducted as part of the NCCI in 1997 was an important first step. It adopted a consensus-based approach that brought together stakeholders from across the full spectrum of health care in the area of cancer control. Importantly, there was an attempt to relate priority judgements to the aims and objectives of cancer control in Australia at the time. While the translation of these aims and objectives into specific criteria can be criticised¹¹, the specification of criteria was a clear signal of the intent to develop an open and transparent process where the rationale for decisions was specified. The discussion in Section 7 illustrates, however, that improvements are possible in meeting this objective.

The NCCI also made attempts to inform participants in its survey and associated ranking processes. The provision of descriptive information on health care system costs and the health burden, for example, undoubtedly helped participants in the stakeholder questionnaire understand the size of the problems being addressed. It is questionable, however, whether this descriptive information was the most appropriate information to guide judgements on resource allocation issues. Arguably, the more central issues for resource allocation are information on the efficacy of the various interventions in reducing the disease burden, the net cost of the interventions and whether the interventions represent value-for-money. While information on efficacy and efficiency may have been canvassed in the NCCI process, it is difficult to assess what role such information played in the decisions taken.

Apart from the type of information provided to participants in priority setting exercises, there is also the related problem of how such information is meant to be utilised. This is difficult to control in a survey of over 600 participants, but is much more amenable to discussion and agreement in a smaller group of representative stakeholders. It is not clear in the NCCI documentation how the

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These criticisms include issues like:

- whether participants had a common understanding of the criteria;
- whether they gave them similar weight in arriving at their ratings/ judgements;
- the extent of stakeholder involvement in the development of the criteria; and
- whether the criteria were consistently applied at the various stages of the filtering process as the original 276 options were reduced to 147, then to 30, to 21 and finally to the 13 chosen for priority action.

State/Territory workshops used the information at their disposal to reduce the number of proposals to 21. Consensus was clearly the dominant theme.

Finally, as a consensus-based approach, the NCCI did not seek to exclude any relevant perspectives or disciplines from the process – quite the opposite was true. It is questionable, however, whether the discipline of health economics (whose primary mission is to assist with choices involving resource allocation) was involved early enough or in a meaningful way.

The PBMA trial was undertaken in response to such criticisms, to see whether or not the NCCI priority setting process could be improved. More specifically, the Working Party was asked to consider whether the PBMA approach was an appropriate technique to include in the priority setting and planning process - and if it was – how could it best be undertaken and utilised?

9.2 How Well Did the PBMA Approach Adopted Work?

The key characteristics of the PBMA approach adopted were:

- a focus on marginal analysis;
- an evidence-based approach with a research team bringing together the best available data;
- the choice of the DALY as the health gain measure;
- a two-stage approach to the assessment of benefit involving both “technical” (i.e. cost per DALY) and “judgement” aspects (i.e. the second stage filters); and
- a macro economic approach to the economic evaluations.

The merit of the PBMA approach was discussed on several occasions in the various meetings of the Working Party. The clear view that emerged from these discussions is that while there were aspects of the PBMA methods and process that could be criticised and/or improved upon (see below), the PBMA approach trialed represented a “*quantum leap forward in the quality of information available for decision-making*” (Professor Mark Elwood). Both the information base (i.e. the briefing papers) together with the process by which the deliberative judgements were achieved, were viewed in a very positive light.

More specifically, the use of an evidence-based approach facilitated by a suitably qualified research team assembling information on efficacy and efficiency was strongly supported. The type and quality of information, the method of its collection and presentation, and the clarity as to its intended use were all important improvements over the NCCI priority setting process of 1997.

The emphasis of the PBMA approach on achieving a clear concept of benefit was also supported. While members saw this discussion and its translation into clear measurement techniques as a challenging process, it was nonetheless accepted as an important task. While there were reservations by some members on aspects of the benefit measurement process adopted (eg. an

over-emphasis on quantitative methods as opposed to qualitative methods; the validity of the disability weights in the Years Lived with Disability (YLD) component of the DALY), the two-stage process was deemed appropriate and a sensible way to proceed. It was also recognised that these were technical issues that could be varied without taking away from the over-arching PBMA approach in which they were employed.

At a more detailed level, the PBMA trial was also attempting to test issues such as:

- the ability of PBMA to deal with quite divergent options in the disease pathway from prevention through to palliation;
- its ability to measure and weight benefits involving multiple dimensions and different levels of evidence;
- its integration of both “technical” and “consensus” approaches to priority setting;
- its ability to break down priority setting into manageable tasks; and
- its likely acceptability to stakeholders.

While time prohibited all the intended options for change from being assessed, the trial nonetheless provided a sound basis for assessing whether the evidence-based approach to PBMA is feasible and whether it constitutes a sensible approach to priority setting. The options covered health promotion, illness prevention, diagnosis and care components of the disease pathway, as well as both mortality and morbidity dimensions of health gain. Members of the Working Party concluded that the PBMA process performed well in relation to these issues and showed promise of being an important addition to the strategic planning process.

9.3 What Could Have Been Done Better?

A key lesson that emerged from the trial is that an evidence-based approach takes time and resources to do properly. If an evidence-based approach (whether PBMA or some other variant) were to be institutionalised into the strategic planning process, then it would be unrealistic to expect the research team to accomplish on a regular basis what was accomplished for the trial. The size and composition of any future research capacity to support future PBMA studies is an important issue for consideration. While health economics and epidemiological skills would be important, other skills might need to be added, particularly if there was a desire to add qualitative assessment to the evidence base gathered.

Similarly, integrating any future PBMA studies into a more realistic time frame should be an important component of future planning. PBMA is essentially an iterative process that seeks to answer the question of whether resources are currently being used to maximise outcomes. This involves the routine generation and assessment of increments and decrements to current services. It also suggests that routine linkages could be established between key data sets that

might feed into a PBMA-styled planning process. This would include the health system cost data (i.e. the MEEM / DCIS data published by the AIHW); the DALY data sets; and any Cochrane-styled data sets on intervention efficacy.

A related issue concerns the demands on any Working Party that may be constituted to participate in PBMA evaluations. For the trial these were greater than what could be expected in routine application. To some extent this reflected the fact that the research team was “feeling its way” with a new approach and was under-resourced for the size of the research task it had attempted. In routine application the Working Party could not be expected to meet so often, and could reasonably expect the materials produced to be more geared to the demands of busy people. As the assessment processes become more routine (eg. assembling the epidemiological evidence; using the DALY and associated disease model; developing the cost estimates; writing the economic protocol; etc) then the Working Party could expect to see far less iterations of the briefing papers. On the other hand, it needs to be remembered that by its very nature the PBMA process places important tasks in the hands of the Working Party (i.e. the PBMA methods to be adopted; the program structure and budget; the selection of options for evaluation; the concept of benefit and associated measurement approach; etc).

The next most significant improvement would be to ensure that more careful thought was given to the involvement and support of the consumer representative on the Working Party (and of the consumer perspective). The late inclusion of a consumer representative on the Working Party in the trial, together with the short time frame available for the study, restricted the integration of a consumer perspective into the methods adopted. While this is a result of the particular circumstances of this study, rather than of PBMA per se, it is nonetheless an important aspect to consider. Involvement from day one, time to constitute and/or tap into consultative networks, support to understand some of the technical material encountered, would all facilitate this. Although the Working Party sought to capture a consumer view in its approach, it was restricted because of the above-mentioned constraints.

A related issue concerns the question of whether or not the Working Party satisfactorily captured all relevant stakeholder interests. While the Working Party was appropriate for a trial study, any more routine application would need to give careful consideration to this issue.

The final issue of significance was the treatment of equity. It would have been the clear preference of the Working Party for equity to be incorporated as a weight to the “cost per DALY” result. As explained in Section 5, however, this was not possible in the time available for the trial. This was viewed as an important research activity for incorporation in any ongoing application of the PBMA approach.

9.4 Broader Issues

One of the important findings to come from the trial of an evidence-based PBMA approach, is that it has the potential to be an important component of the planning process for cancer control in Australia. The exact nature of that involvement requires further careful thought. Issues to be covered here include:

- who would organise/undertake any ongoing PBMA work program, including its integration with CSG and NCCI activities?
- how would adequate resources be gathered to fund any ongoing PBMA program?
- how would PBMA have been integrated into the 1997 NCCI exercise (i.e. at what points in the process could it have been integrated)?
- what are the strengths and weaknesses of the DALY as the prime measure of health gain?
- what are the research and data implications of an ongoing PBMA program of work (eg. development of equity weighting; DALY disaggregated into target groups; maintain the AIHW DCIS data base; develop the efficacy / effectiveness data base)?

are there issues in consistency of priority setting approaches across the priority disease areas (including the issue of common risk factors and the potential to share key data bases)?

is the information on the second stage filters being appropriately utilised (i.e. do we leave as judgement process or attempt to integrate into one benefit score using decision theory)?

should the program structure and program budget aspects of PBMA be activated? and

how would the PBMA work be integrated with any broader consultation?