The Auditor-General

The Pharmaceutical Benefits Scheme

Department of
Health and Family Services

Australian National Audit Office

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Canberra ACT
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Dear Madam President
Dear Mr Speaker

In accordance with the authority contained in the *Audit Act 1901*, the Australian National Audit Office has undertaken a performance audit in the Department of Health and Family Services and I present this report and the accompanying brochure to the Parliament. The report is titled *The Pharmaceutical Benefits Scheme*.

Yours sincerely

P. J. Barrett
Auditor-General

The Honourable the President of the Senate
The Honourable the Speaker of the House of Representatives
Parliament House
Canberra ACT

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Abbreviations / Glossary

AAT	Administrative Appeals Tribunal
ADEC	Australian Drug Evaluation Committee. This Committee has an important role in TGA's work.
AFAO	Australian Federation of AIDS Organisations
AMA	Australian Medical Association
ANAO	Australian National Audit Office
APAC	Australian Pharmaceutical Advisory Council
APMA	Australian Pharmaceutical Manufacturers Association
ARTG	Australian Register of Therapeutic Goods
CEO	Chief Executive Officer
CHF	Consumer Health Forum
cohort	a group of submissions arriving before the deadline for a particular PBAC meeting which, under its own procedures, DHFS has guaranteed to ensure are considered at that PBAC meeting - thus avoiding build up of a backlog of applications.
comparator	the drug already on the PBS Schedule with which a sponsor's proposal for listing is compared. The comparator is usually the drug most frequently used for the treatment of the indication which the new drug is targeting
DHFS	Department of Health and Family Services
DHSH	Department of Human Services and Health
DIST	Department of Industry, Science and Tourism
DoF	Department of Finance
DUSC	Drug Utilisation Sub-Committee of the PBAC
ESC	Economic Sub-Committee of the PBAC
EU	European Union
generic drug	an alternative brand of an out of patent pharmaceutical product
GP	General Practitioner
HIC	Health Insurance Commission
HIV-AIDS	Human Immuno Deficiency Virus, that when it is advanced becomes Acquired Immuno Deficiency Syndrome, AIDS
HSD	Health Services Division (DHFS)
IC	Industry Commission
indication	the disease or disorder which is the reason for commencing therapy

MHFS	Minister for Health and Family Services
MS	multiple sclerosis
NCE	new chemical entity
PBAC	Pharmaceutical Benefits Advisory Committee
PBB	Pharmaceutical Benefits Branch (HSD, DHFS)
PBPA	Pharmaceutical Benefits Pricing Authority
PBS	Pharmaceutical Benefits Scheme
PBS Schedule	the PBS Schedule is the comprehensive listing of drugs and medicinal preparations subsidised by the Government under the Pharmaceutical Benefits Scheme
PES	Pharmaceutical Evaluation Section (PBB,HSD,DHFS)
PHARM Committee	Pharmaceutical Health and Rational use of Medicines
PRS	Prices and Remuneration Section (PBB, HSD, DHFS)
RACGP	Royal Australian College of General Practitioners
the Schedule	see ‘PBS Schedule’ above
sponsor	the firm that proposes a pharmaceutical product for listing on the PBS (usually the manufacturer or importer)
TGA	Therapeutic Goods Administration
therapeutic group used to	a group of drugs which act in a similar way and are
treat similar conditions	
UK	United Kingdom
UNSW	

Part One

Summary and Recommendations

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Summary

Audit Background

1. The Pharmaceutical Benefits Scheme (PBS) was established in 1950 to provide access to life saving drugs to people who otherwise would not be able to afford them. Since then, the purpose of the Scheme has widened to provide timely, reliable and affordable access for the Australian community to necessary and cost effective medicines. In 1996-97, Government expenditure under the Scheme was $2.5 billion and patient co-payments contributed a further $530 million.

2. In 1995, the Australian National Audit Office (ANAO) began a two part review of the Department of Health and Family Services' (DHFS') programs for general marketing approval of pharmaceutical products. The review covers the actions of the Therapeutic Goods Administration (TGA) and of DHFS' selection and purchasing of drugs for the PBS. The first part was completed with the tabling in 1996 of the performance audit report on Drug Evaluation by the TGA. This report covers the second part of this review.

3. In May 1996 the Industry Commission released its report on the pharmaceutical industry in which it recommended, among other things, that the Government undertake a review of the PBS listing process. Subsequently, Dr Wooldridge, the Minister for Health and Family Services wrote to the Auditor General asking him to incorporate the review of the listing process into the already planned audit. The Auditor General agreed to the Minister's request.

Audit Objective

1. The objective of the performance audit of the Pharmaceutical Benefits Scheme was to evaluate the Department's performance in pursuit of selected PBS program objectives and outcomes, including to investigate and evaluate the efficiency, administrative effectiveness and accountability of the management of the listing process as a significant element of the program.

2. This involved a review of the developments in the listing process over recent years including:

- the establishment of a comprehensive database of major applications for PBS listing between 1991 and 1996, which facilitated a detailed analysis of the time taken to list drugs on the PBS schedule;
- a technical consultancy into the DHFS' Guidelines to industry for preparation of applications for PBS listing, and into the use of the economic analysis in assessing proposals for PBS listing; and
- a review of the selection process including the operations of the PBS advisory committees.

Conclusions

Efficiency

1. In 1996-97 $10.1 million in running costs was expended on administration of the PBS listing process. These running costs supported management of the $2.5 billion of government expenditure on pharmaceutical benefits.

2. The time taken to process applications for PBS listing was a key indicator of the efficiency of PBS listing. ANAO noted that:

- since 1991, the Guidelines for the PBS listing of pharmaceutical products administered by DHFS have become far more complex. The Industry Commission has stated that Australia is regarded as being at the leading edge internationally in requiring economic analysis to support the Government's subsidising or purchasing of pharmaceuticals;
- in addition to administering much more complex guidelines, the workload in the PBS process has, over recent years, increased considerably; for example, the number of major applications increased from an average of 36 per year in the period 1991-93 to 58 per year in the period 1994-96. Staffing in PBB, however, has remained relatively stable at between 60 to 65 staff from 1993 to 1996, falling to just under 60 in 1996-97;

- notwithstanding these statistics, the time allowed in the timetable for processing major applications for PBS listing has been reduced from between 170 and 185 working days prior to 1993 to between 145 and 160 working days from 1993 to date;
- the proportion of major applications approved for listing in the minimum time (that is, in a single cycle of the PBS selection process) declined after new requirements for economic analysis were introduced in 1993 to an average of 35 per cent in the period 1993 to 1995. However, the proportion increased to 48 per cent in 1996. This increase in the proportion of major applications accepted for listing in the minimum time has also resulted in further reductions in the average time for applications to be listed; and
- in addition, the average time to achieve PBS listing for major applications which, not being accepted at their first submission for listing, required reconsideration by the relevant DHFS advisory committees, also fell significantly between 1993 and 1996 from over 400 working days to between 220 and 280 working days;
- ANAO concludes that, overall, the Department's management of the PBS listing process was efficient, with significant improvement achieved in recent years on the basis of the above indicators. This report flags further areas that offer scope to achieve some additional efficiency gains.

Administrative effectiveness

1. The selection of drugs for PBS listing involves preparation by the sponsor (ie, the pharmaceutical manufacturer or importer) of an application using DHFS' Guidelines. The applications are considered by professional and expert advisory bodies which make recommendations on listing and price to the Minister, or recommendations to Cabinet, in the case of drugs estimated to cost the PBS over $10 million per annum.

2. The effectiveness of the listing process depends on the quality of the guidance provided by DHFS to pharmaceutical companies, on the quality of the information provided by those companies in their applications, and on the level and soundness of judgement brought to bear in the selection of drugs the Government purchases for provision under the PBS.

3. The ANAO concluded that:

- the progressive introduction since 1991 of an evidence based approach - requiring sponsors to provide data from clinical trials and economic analysis in support of applications for PBS listing - has been a major contributor to the administrative effectiveness of the listing process;
- the Guidelines provided by the Department to industry were soundly based and useful, providing a suitable basis for provision by industry of sufficient evidence to facilitate sound decision making on applications;
- departmental processes, including those of its advisory committees for consideration of pharmaceutical companies' applications for PBS listing, worked effectively; and
- DHFS' selection processes were rigorous and allowed high levels of clinical experience and judgement to be applied to the selection of drugs.

4. The ANAO concluded that, in a program requiring both efficient operation and considerable ongoing technical change and development, DHFS' implementation of Government policy through the PBS has been administratively effective. In this context there remains a range of further necessary developments for improvement involving the need to successfully put them into operation. ANAO has made a number of recommendations and suggestions in this respect which are outlined in the key findings which follow, including:

- measures to improve and better promote the Guidelines to industry;
- technical developments in the use of economic analysis aimed at improving the process of evaluating drugs in terms of their value for money to the PBS; and
- proposals that DHFS better define its strategy to ensure value for money and contain cost escalation in its purchasing of drugs.

Accountability

1. DHFS has followed the Government's guidelines for reporting to Parliament on its performance in administering the PBS;

2. Notwithstanding this adherence, ANAO considers that reporting to Parliament and to stakeholders could be improved to facilitate understanding of the reasons for the selection of pharmaceutical

products being purchased and listed on the PBS. As a result, more realistic expectations of the listing process could be developed among the various stakeholders. DHFS has undertaken to provide performance measures that should allow Parliament and the public to understand better the operations and outcomes of the program than they have in the past.

Recommendations and DHFS response

1. The ANAO made fifteen recommendations aimed at improving the management of the PBS, in particular, the listing process. The Department noted that it appreciated the value of an independent review of the PBS process. Of the fifteen recommendations, twelve were agreed and three were agreed with some qualification.

Efficiency issues

1. The increased DHFS workload since 1993 has imposed some strain on staffing resources at critical stages in the listing process. Further out-sourcing of the evaluation of companies' applications would alleviate this pressure at the peak work-load times and free-up key permanent staff to focus on the important further development of the PBS selection process. Whilst this proposal has resourcing implications, these should be considered in the context of the net potential benefits of the changes and in relation to overall PBS resourcing requirements.

2. Following the major reductions in processing time after the rearrangement of the process in 1993, opportunities for further reducing the average time for PBS listing are now limited. The potential benefits from those restricted opportunities must also be considered against the need to avoid jeopardising the major priorities of maintaining high standards of evaluation of applications, and of ensuring value for money for the Government and fairness to the companies concerned. Further limited reductions in the processing time could be assisted by:

- increasing the proportion of drugs approved at their initial submission to DHFS' advisory committees. This measure depends not only on the efficiency and effectiveness of DHFS' processes, but on the quality of information provided by manufacturers - and ultimately on the cost effectiveness of the drugs in question;
- improved monitoring of the duration of the listing process for individual applications with a view to identifying how the time taken for the listing process could be reduced; and
- working towards producing and distributing the PBS Schedule electronically - as a medium term likely cost effective objective. Currently, printing and mailing of the Schedule in book form to medical practitioners takes ten weeks. A major problem inhibiting more rapid production and electronic distribution of the Schedule is the relatively low level of computer use by medical practitioners.

3. In order to optimise the benefits of out-sourcing the evaluation of sponsors' applications for PBS listing, DHFS should promote greater competition by seeking expressions of interest from the increasing

number of institutions capable of performing the evaluation role for more cost effective outcomes.

Administrative effectiveness issues: the process for selection of drugs for PBS listing

1. ANAO found that the Guidelines set high standards of evidence required to support applications. The Guidelines provided a useful handbook to assist sponsors in presentation of both clinical and economic information.

2. The shared opinion of the advisory committees, ANAO's consultants and of a majority of industry representatives interviewed by the ANAO is that the Guidelines have facilitated improvement in the quality of sponsors' submissions and that the usefulness of the information in applications has improved considerably. However, the quality of information varies across applications. There is room for improvement in industry compliance with the Guidelines, especially in respect of the quality of the economic analysis and, in particular, the assessment of financial outcomes of adding a drug to the PBS schedule. Only three of the thirty applications in the ANAO sample of applications were defect free, nine applications had one defect, eight had two defects, seven had three defects, and three had four defects.

3. Industry views on the PBS listing process varied considerably. The majority of companies interviewed accepted the rationale for the evidence-based approach and the use of economic analysis in, and as applied to, their submissions. However, many had reservations about aspects of the process, including among others things, the complexity of the Guidelines used for making submissions for PBS listing and the transparency of the listing process. A minority of companies was quite critical of listing processes. These companies considered DHFS' information requirements unique to Australia and onerous for such a small market.

4. In order to maintain their efficacy and efficiency for industry and for the DHFS' advisory committees, the Guidelines would benefit from incremental change as improved techniques for economic analysis are accepted rather than making a major change every three to four years.

5. DHFS could provide more assistance with the Guidelines to less experienced sponsors to help them comply more quickly, for example, by more interaction at the early stages of their submissions.

6. Once sponsors submit their major applications, DHFS should increase interaction with them on complex proposals with the aim of clarifying details and removing potentially minor pitfalls before consideration by the advisory committees.

7. The selection process should give greater emphasis to economic analyses of companies' applications and to value for money considerations, while maintaining the importance of clinical benefits from use of the drugs, as provided for in the relevant legislation.

8. The current emphasis on cost effectiveness analysis provides for comparison between drugs treating similar indications[1] such as, for example, hypertension, but does not provide a basis for comparing value for money of drugs treating different indications, such as hypertension and depression. Several technical developments in the economic analysis of applications for listing could assist in developing more universal outcome measures (from treatment following use of drugs) to allow comparison of the value to the health system of drugs addressing different indications or medical problems. These may include

- the encouragement of and a wider use of cost-benefit analysis;
- more effective use of sensitivity analysis in economic analysis; and
- better articulation and integration of qualitative measures of intangible benefits (such as, for example, equity considerations) with more readily quantifiable measures in economic analysis.

9. In order to reduce industry confusion about the significance that can be attached to offsetting savings associated with possible listing of a drug, DHFS should clarify the extent to which 'non-pharmaceutical' savings can be considered permissible and hence given due weight in the decision making process. For example, when patients taking a particular drug do not require hospitalisation, sponsors sometimes argue that the cost of a hospital bed is saved and that this should be acknowledged as an offset to the cost of the drug. However, in many cases, such savings (and consequent benefits) would be limited to reduction in the next hospital patient's waiting time for admission (important though that may be to the person concerned).

[1] That is, the disease or disorder which is the reason for commencing therapy.

10. ANAO found that DHFS and the industry representative body promoted greater transparency in the listing process than had been the case in the recent past. DHFS' peak advisory body, the Pharmaceutical Benefits Advisory Committee (PBAC), meets regularly with the peak industry representative body. DHFS had frequent written and face to face contacts with industry representatives.

11. The composition of advisory bodies indicated little change over a number of years. Following the 1993 requirement for cost effectiveness data in sponsors' applications for listing, and the consequent need for judgement on economic issues, DHFS should now consider whether the composition and operations of the advisory bodies are consistent with their changing roles to ensure the best possible advice.

Administrative effectiveness issues: PBS as a purchasing program

1. Government spends over $2.5 billion annually on PBS. The cost of the program has grown at between eight and thirteen percent per annum in real terms in recent years. Although the provision of drugs and medicines operates through a subsidy system, the PBS is very much about negotiating prices and purchasing pharmaceutical benefits from pharmaceutical manufacturers.

2. An examination of all the factors affecting growth in Commonwealth outlays under the program was beyond the scope of this audit. It did, however, focus on the listing process as one significant factor.

3. The particular Budget status of PBS bears on DHFS' management of the program. Rather than having a Budget limit predetermined as is the case with most appropriations, PBS has a Special Appropriation and is demand driven. In such a case, the Budget outcome each year depends on the level of demand by consumers for the goods provided by the Scheme. This makes some conventional purchasing and budget management operations, especially in setting priorities and planning purchasing strategies, difficult to apply and operate effectively.

4. In addition, DHFS could achieve greater value for money if it were in a better position to more fully promote to the public and to GPs the quality use of the medicines which it subsidises. The secrecy provisions of the National Health Act have the incidental effect of imposing limitations on how far DHFS can advise the public and medical practitioners of the relative merits of the drugs which it has purchased

through PBS. DHFS should examine whether the relevant sections of the Act are still necessary to effectively implement Government policy, and if not, what changes to the Act would be desirable to allow prescribers and users to be better informed about the benefits, limitations and costs of drugs available through the PBS.

Recommendations

Recommendation 1

ANAO recommends that DHFS maintains an integrated database to monitor the progress of applications through the PBS listing process, and to provide relevant statistics on the efficiency of the listing process for management and for reporting to the Government, Parliament and major stakeholders.

DHFS Response:

Agreed.

Recommendation 2

ANAO recommends that DHFS explores ways to reduce the average time taken to list drugs on the PBS insofar as this is consistent with rigorous evaluation and value for money, through avenues such as:

- avoiding delays to correct relatively minor inadequacies in sponsors' applications for (PBS) listing;
- increasing the proportion of applications accepted for listing in the first cycle of evaluation;
- more effectively using IT resources to support operation of the listing process; and
- reducing the time taken to produce the PBS Schedule.

DHFS Response:

Agreed with qualifications.

Recommendation 3

To improve the quality of the economic analysis required of sponsors in submissions for PBS listing, the ANAO recommends that, in its revision of the Guidelines for the Pharmaceutical Industry on Preparation of Submissions to the PBAC, DHFS consider incorporating into the Guidelines a number of technical developments, involving among other things:

- the more discriminating use of sensitivity analysis;
- better articulation and integration of qualitative assessments of intangible benefits (eg, equity considerations) with quantifiable measures;
- the more frequent use of cost-benefit analysis; and
- the development of more uniform outcome measures to allow comparison of the value for money to the health system of drugs addressing different indications or medical conditions.

DHFS Response:

Agreed.

Recommendation 4

ANAO recommends that DHFS has in place a procedure to record the strengths and weaknesses of the use of cost effectiveness analyses in individual sponsor applications in order to guide its advice to industry.

DHFS Response:

Agreed.

Recommendation 5

ANAO recommends that DHFS increases its promotional efforts and guidance to less experienced sponsors of new drugs in the PBS listing process to allow these sponsors to more quickly comply with the Guidelines and provide information of high quality.

DHFS Response:

Agreed.

Recommendation 6

ANAO recommends that DHFS considers initiating more effective face-to-face consultation with companies following initial assessment of their more complex submissions, in order to:

- provide companies with more knowledge of the listing process; and
- clarify as many issues and data requirements as possible before they are provided to the Department's advisory committees.

DHFS Response:

Agreed.

Recommendation 7

ANAO recommends that DHFS gives greater priority to the revision of the Guidelines for the Pharmaceutical Industry on Preparation of Submissions to the PBAC so that technical improvements currently being considered can be quickly refined and be integrated into the selection process and that these improvements be added to the Guidelines progressively rather than waiting until 1999 to prepare a new edition.

DHFS Response:

Agreed with qualifications.

Recommendation 8

ANAO recommends that DHFS clarifies, in the Guidelines for the Pharmaceutical Industry on Preparations of Submissions to the PBAC, the context and the extent to which sponsors, in their submissions to PBAC, can use data on potential cost savings elsewhere in the health system, such as in hospitals, through the effective use of pharmaceutical products.

DHFS Response:

Agreed.

Recommendation 9

ANAO recommends that the PBPA should be provided with additional economic and pricing analyses and data to better inform its decision making.

DHFS Response:

Agreed with qualifications.

Recommendation 10

ANAO recommends that DHFS reviews the roles and composition of PBS advisory committees to ensure that, in addition to the present high level of consideration of clinical and pharmaceutical issues, the best use is made of economic data in applications for PBS listing.

DHFS Response:

Agreed.

Recommendation 11

ANAO recommends that DHFS develops its systematic monitoring of the use and the total cost of pharmaceuticals on the PBS in order to establish whether the basis on which particular prices were agreed with manufacturers remains valid.

DHFS Response:

Agreed.

Recommendation 12

ANAO recommends that DHFS explores ways in which the Commonwealth can better inform prescribers and users of the benefits, limitations and costs of the drugs available through the PBS.

DHFS Response:

Agreed.

Recommendation 13

ANAO recommends that DHFS considers ways to strengthen the roles of advisory committees in advising the Minister on the cost implications of total PBS listings, by making this requirement more specific.

DHFS Response:

Agreed.

Recommendation 14

The ANAO recommends that the DHFS better inform industry and the public about the PBS listing process in order to reduce misconceptions about the role of the Department in this process, and facilitate understanding of the reasons behind the Department's purchase of pharmaceutical products.

DHFS Response:

Agreed.

Recommendation 15

The ANAO recommends that, in order to take advantage of the growing number of institutions capable of fulfilling the evaluation role, the DHFS broadens the competition for provision of evaluation advice to the Department on cost effectiveness data provided by pharmaceutical companies on their products.

DHFS Response:

Agreed.

Part Two

Audit Findings and Conclusions

1. Introduction

Background to the audit

1.1 In 1995, the Australian National Audit Office (ANAO) began a two part review of the evaluation and purchasing of pharmaceutical products by the Department of Health and Family Services (DHFS). The first step was completed in 1996 with the tabling of Audit Report No. 8 of 1996-97, *Drug Evaluation by the Therapeutic Goods Administration, Department of Health and Family Services*. TGA's objective is to ensure not only that the safety, quality and efficacy of therapeutic goods available in Australia is at a standard equal to that of comparable countries, but also that pre-market assessment of therapeutic goods is conducted within a reasonable time. Within this program, the TGA is responsible for the evaluation and approval of pharmaceutical drugs for marketing to the public.

1.2 In early 1996, ANAO advised DHFS that it proposed to conduct a performance audit of the Pharmaceutical Benefits Scheme (PBS). This performance audit was to be the second part of the review.

1.3 In May 1996, the Industry Commission (IC) released its report on the pharmaceutical industry. One of its recommendations was that the PBS listing process be subject to a review[2]. The Minister for Health and Family Services (MHFS), Dr Wooldridge, wrote to the Auditor-General in September that year asking him to incorporate the review of the listing process into the already planned audit. The Auditor-General agreed to the Minister's request.

1.4 The PBS was established in 1950 to provide access to life-saving drugs to people who otherwise would not be able to afford them. Initially, 139 pharmaceutical products were listed. Since then the purpose of the Scheme has widened to provide timely, reliable and affordable access for the Australian community to necessary and cost effective medicines. At present just over 1800 products[3] are listed in the current Pharmaceutical Benefits Schedule. In 1996-97, Government

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[2] Industry Commission, Report No.51, 3 May 1996, *The Pharmaceutical Industry*, AGPS, Canberra, 1996, Recommendation 4.

[3] *Schedule of Pharmaceutical Benefits, For Approved Pharmacists and Medical Practitioners* Department of Health and Family Services, August 1997, p.2.

expenditure under the Scheme was $2.538 billion[4] and patient co-payments contributed a further $530 million. Government expenditure under the Scheme for 1997-98 is estimated at $2.820 billion.

Once a pharmaceutical product is listed on the PBS Schedule, the Government agrees to subsidise the product's cost. Patients are required to make a co-payment. In 1997-98, the patient co-payment is a maximum of $20 per prescription. Social security and other beneficiaries may be eligible to make a smaller patient co-payment, called a concessional co-payment. In 1997-98, this concessional co-payment is $3.20 per prescription.

The Commonwealth Government pays to the pharmaceutical companies the remaining cost of its products above the relevant patient co-payment in order to keep their prices affordable. In 1996-97, for instance, the Commonwealth's smallest payment for a pharmaceutical product listed on the PBS Schedule was $2.23 per prescription, while the largest Commonwealth payment for a listed pharmaceutical product was $4,720 per prescription. The average of all Commonwealth payments under PBS in 1996-97 was $18.90 for each prescription.

The Commonwealth Government uses the market power achieved through its granting of product subsidies under the PBS to hold down prices paid to producers for listed drugs and hence the PBS costs[5]. The Commonwealth's influence over the Australian market for pharmaceutical products is considerable. In fact, it approaches a monopsony[6] power.

1.2 Objective and scope of the audit

The objective of the performance audit of the PBS was to evaluate the Department's performance in pursuit of selected PBS program objectives in particular to investigate the efficiency, effectiveness and accountability of the administration of the listing process. Emphasis was on those objectives and outcomes more closely related to the listing process as a significant element of the program.

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[4] *Portfolio Budget Statements 1997-98, Department of Health and Family Services*, AGPS, Canberra 1997, p 135-6.

[5] Industry Commission, 1996, p 76.

[6] A monopsony is a situation where there is a dominant purchaser in a market with multiple sellers.

1.3 Audit methodology

The audit has involved four main methods of enquiry:

a. interviews with representatives of key stakeholders including:

- officers of the Department of Health and Family Services;
- the chairpersons and some members of the Pharmaceutical Benefits Advisory Committee (PBAC) and its Economic Sub-Committee (ESC), and of the Pharmaceutical Benefits Pricing Authority (PBPA) - these interviews were supplemented by ANAO attendance at meetings of these committees ;
- chief executive officers (CEOs), and/or senior executives of 16 major pharmaceutical companies accounting for approximately 70 per cent of major pharmaceutical products listed on the PBS; and
- representatives of consumer groups, professional bodies and other interested parties.

b. development by the audit team of a computerised database to track the progress of PBS applications, based on DHFS Pharmaceutical Benefits Branch (PBB) data. The database lists all 386 major applications submitted from 1991 to 1996 and 268 minor applications lodged since early 1995. Comments in this report relate to analysis of major applications only[7];

c. the use of consultants to analyse some technical issues, notably the use of cost effectiveness analysis in the listing and selection process, and an econometric analysis of the factors affecting listing and pricing; and

d. a review of relevant DHFS documents and records of the PBS.

Fieldwork was conducted between December 1996 and June 1997.

Audit criteria are the means by which audit outcomes are to be judged. As part of the audit, criteria were developed to consider if:

- DHFS had efficient administrative processes for the listing of pharmaceutical products on the PBS;

[7] The significant differences between major and minor applications are defined and discussed in Chapter 2.

- DHFS had effective processes for listing pharmaceutical products;
- DHFS could demonstrate effective accountability arrangements for the administration of PBS to the Government, Parliament, industry and consumers; and
- PBAC was acting in accordance with its role.

In April 1997, about mid-way through the audit, the ANAO released a discussion paper to the Minister and to the Department on the issues then identified for comment.

The two sets of consultants employed by ANAO during the audit were:

- Mr Geoffrey Dixon and Dr Geoffrey Vaughan, who completed a study of the use of economic analysis of the PBS listing process; and
- the Australian Bureau of Statistics, ABS, which completed a preliminary econometric analysis of the factors affecting the listing and pricing of pharmaceutical products.

The audit was conducted in accordance with ANAO Auditing Standards. It cost
$444 000.

2. THE LISTING PROCESS IN OUTLINE

2.1 Introduction

The Commonwealth Government provides pharmaceutical benefits (ie, drugs and medicinal preparations) under the Pharmaceutical Benefits Scheme (PBS). This provision is publicly notified by listing of the drug in the (PBS) Schedule which is published by DHFS every three months.

PBS listing involves a two-part process:

- as prerequisite, manufacturers or importers (the 'sponsors') of the pharmaceutical product must obtain marketing approval from the Therapeutic Goods Administration (TGA) which tests drugs for safety, quality and efficacy;

- the sponsor must then apply for the drug to be added to the PBS Schedule; this involves an evaluation and selection process, central to which is advice to the Minister by the Pharmaceutical Benefits Advisory Committee (PBAC) and the Pharmaceutical Benefits Pricing Authority (PBPA). The advice includes whether the drug should be subsidised under the PBS, and for what purposes and within what limitations if necessary.

Manufacturers or importers usually apply for a listing under the PBS for most prescription pharmaceutical products approved by the TGA. This is because, without the Commonwealth subsidy under the PBS, drugs would be unlikely to achieve sales that would provide pharmaceutical companies with an adequate return in Australia on their investment.

The implications of the Commonwealth Government's role as provider and proxy purchaser of drugs is discussed in Chapter 5.

2.2 Description of the PBS process

The objective of the PBS, as stated in the Portfolio Budget Statements for 1996-97, is to provide timely, reliable and affordable access for the

Australian community to necessary and cost effective medicines[8]. The PBS goals in that year were to ensure that:

- the Pharmaceutical Benefits Schedule includes all necessary medicines, reflecting modern and appropriate treatment;
- the listing processes and containment of the cost of the Scheme are efficient; and
- there is acceptable quality and cost effective use of medicines.

Drug application and listing process

Pharmaceutical companies apply to the DHFS through the Pharmaceutical Benefits Branch (PBB) to have their products listed in the PBS Schedule. The PBB analyses the applications and provides secretariat services to the Pharmaceutical Benefits Advisory Committee (PBAC), and to the Pharmaceutical Benefits Pricing Authority (PBPA). The PBAC is established under Section 100 of the *National Health Act 1953* to make recommendations to the Minister for Health about which drugs and medicinal preparations should be available as pharmaceutical benefits, and to advise the Minister on any other matter relating to the PBS which is referred to it by the Minister. PBPA advises the Minister on pricing matters. The process from receipt by the PBB of an application for listing of a pharmaceutical product, to final inclusion in the Schedule, takes about 8 calendar months.

There are several categories of drug applications summarised under 'major' and 'minor' submissions as follows:

- major submissions are for:

  - listing new drugs;

  - a significant change to a current listing;

  - a review of the comparative cost-effectiveness of a currently listed drug in order to change a PBAC recommendation to the PBPA on its therapeutic relativity or price premium;

[8] *Portfolio Budget Statements 1996-97, Health and Family Services Portfolio*, AGPS, Canberra 1996, p119. Financial year 1996-97 is the year in which the audit was planned and conducted and hence more relevant to the audit than 1997-98 when the audit was tabled. Some significant modifications of goals and priority outcomes between the two financial years are discussed in Chapter 5.

- a new formulation of a currently listed drug for which a price premium is requested by the sponsoring pharmaceutical company; and

- re-submissions by sponsors of applications previously rejected by the PBAC.

- minor submissions include applications where:

  - an economic evaluation is not required, for example, a change to the maximum quantity or a change to the number of repeat prescriptions;

  - new formulations are available at the same or lower relative price to currently listed formulation; and

    - new strengths of drugs or changes to an indication/restriction are proposed (when based on clinical considerations only and no cost effectiveness considerations).

Submissions for listing of generic equivalents (or new brands) of an already listed drug, while not considered by the PBAC, are dealt with by the Pharmaceutical Benefits Branch.

Major submissions are subject to all PBS processes (notably the PBAC and its Economic Sub-Committee (ESC) and PBPA consideration), while minor submissions are subject to a more limited number of processes (excluding the ESC and, in some circumstances, the PBAC).

The PBS listing process for major submissions is depicted in Table 2.1 and at Figure 2.1, in which the duration of time is shown sequentially from the cut-off date for applications to a particular PBAC meeting.

The full listing process involves consideration by the expert committees - the PBAC and ESC and by the PBPA, with final approval by the Minister, or, if the projected Commonwealth expenditure on a newly listed product is likely to exceed $10m. per year, by the Cabinet. The main elements of the listing process are as follows:

- receipt by the PBB of a pharmaceutical company's application for listing of a pharmaceutical product. Applications are prepared by the company in accordance with the Guidelines for preparation of

submissions to PBAC provided by DHFS.[9] The cut-off date for receipt of submissions is usually 11 weeks before the particular PBAC meeting. Currently, meetings are held in the first week of March, June, September and December;

- evaluation by the Pharmaceutical Evaluation Section (PES). The PES reviews and interprets the economic analyses in sponsors' submissions and provides the results of the review together with sponsors' submissions to the ESC for its consideration. The latter occurs about eight and a half weeks after the cut-off date for submissions, and some 2 to 3 weeks before the PBAC meeting. The PES evaluation is provided to the sponsor before the PBAC meeting, which gives the sponsor the opportunity to comment to PBAC on the PES analysis;

- about 11 weeks after DHFS has received a sponsor's submission the PBAC considers it and the advice received from the ESC along with the sponsor's rejoinder (if any). If approved, the application moves to the next stage for PBPA consideration. Rejected applications progress no further than PBAC. Sponsors can resubmit applications to a later meeting with additional information, or not. Sponsors may resubmit applications as often as they like.

- after the PBAC meeting, the PBB's Prices and Remuneration Section (PRS) prepares information relevant to the PBAC recommendation on the product's price for consideration by the PBPA, which meets usually five weeks after the PBAC. The PBPA decides on a preferred price or price range in relation to which the PRS negotiates, with sponsors, a final price for the new listing in the Schedule;

- the PBAC recommendation with the agreed price is then referred to the Minister for Health or, for drugs with estimated annual cost over $10 million, to the Cabinet for approval;

- PBB then undertakes production, publication and despatch of the Schedule to medical practitioners and approved pharmacies. This process currently takes around 10 weeks; and

- overall, the minimum time from a sponsor's application to listing pharmaceutical product is 32 weeks.

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[9] *Guidelines for the Pharmaceutical Industry on Preparation of Submissions to the Pharmaceutical Benefits Advisory Committee.* Department of Health and Family Services, 1995, Canberra.

Table 2.1 Stages in the PBS listing process

Function	Steps in the PBS listing process
1. Preparation by the sponsor of a submission in accordance with Guidelines for the Pharmaceutical Industry on Preparation of Submissions to the Pharmaceutical Benefits Advisory Committee (The Guidelines). The Submission can be lodged prior to TGA ADEC's consideration for marketing approval.	
2. Receipt by the PBB of the sponsor's application	beginning of DHFS' processes
3. Economic Evaluation by the PBB's Pharmaceutical Evaluation Section (PES)	
4. Provision of Economic Sub-Committee agenda to ESC members	7 weeks after receipt
5. PES' economic evaluation is sent to the PBAC Secretariat, and the PES's evaluation and PBAC secretariat overview is provided to sponsors	8 weeks after receipt
6. PBAC agenda provided to members	8.5 weeks after receipt
7. **ESC meeting to evaluate data on the comparative cost-effectiveness of drugs proposed for listing**	**8.5 weeks after receipt**
8. Pre PBAC comments provided by sponsor	9.5 weeks after receipt
9. ESC reports and sponsor comments are provided to PBAC members	10 weeks after receipt
10. **PBAC meeting**	11 weeks after receipt
11. PBAC Secretariat provides oral information on PBAC decisions to sponsors	the first working day after PBAC meeting
12. PBAC Secretariat provides written	14 weeks after receipt

advice to sponsors	
13. **PBPA meeting**	**15-17 weeks after receipt**
14. Approval for listing is given by the Minister (or by the Cabinet if annual cost over $10 million)	around 21-23 weeks after receipt
15. Samples of drugs are assayed by the TGA to check that products comply with the required standards. For example, a tablet is tested to ensure that the active ingredient is identical to that stipulated by the sponsor	
16. Publication and dispatch of the Schedule to approved pharmacies and medical practitioners	approximately 32 weeks after submission received by PBB

Insert Figure 2.1

PBB staff provide support to the PBAC and its sub- committees. This support comes in the form of:

- PES's provision of economic evaluations and secretarial assistance to the ESC and advice to PBAC;
- the PBAC Secretariat and Listing Section's provision of secretariat support and provision of summaries of drug evaluations to the PBAC; and
- PRS's provision of secretariat support and recommendations on prices to the PBPA.

2.3 Differing stakeholder perceptions of the PBS

In order to more fully understand the opinions and suggestions of different stakeholders encountered in the course of the audit, the ANAO sought to assess how they perceived the PBS. This section summarises the results of the ANAO's assessment gained mainly through discussion with stakeholder representatives. Stakeholders include the public both as consumers of drugs and as taxpayers paying for the PBS benefits, the Government (as manager of PBS and proxy purchaser of drugs), health professionals, their representative bodies and the pharmaceutical industry.

There are widely differing views on the value of the PBS as a means of providing subsidised pharmaceuticals to the public. On the one hand, industry and consumer representatives provided a generally favourable impression of the PBS as a program, but there were some strong criticisms of certain aspects of the listing process from certain sections of the industry. In similar vein to those strong criticisms, the 1996 Industry Commission report concluded that the PBS was the major obstacle to the development of the pharmaceutical industry[10].

There appeared, however, to be a general view that the PBS was fundamentally a good scheme. This was summarised by a CEO of one major multi-national company in these terms : `broadly speaking the current system of subsidies for pharmaceuticals works well; the health of all Australians is good compared to other countries where in many cases it is mainly the rich who have access to medicines.'

[10] Industry Commission, 1996, p XXIX.

The same CEO noted that `in countries where drugs are subsidised, pharmaceutical businesses are comparatively large. In this sense Australia is a good country for the pharmaceutical companies. Listing on the PBS Schedule ensures large industry sales and availability of health care to all Australians. However, companies are struggling to survive and fighting to keep the manufacturing going.'

A majority of industry representatives tended to share this qualified but overall positive view about the benefits arising from the PBS. This was in contrast to the generally negative industry views of the PBS referred to in 1996 Industry Commission report[11].

Several industry representatives agreed that Australia was not `a bad market' for pharmaceutical companies. Some commentators noted that no pharmaceutical manufacturer had withdrawn from the Australian market because of the PBS, and, in an environment of world wide rationalisation of pharmaceutical manufacturing plants, comparatively few companies had ceased manufacturing in Australia.

The negative industry view was put by CEOs and senior executives of a number of other pharmaceutical companies, who argued that the PBS reduced the likelihood of health consumers gaining rapid access to the most modern pharmaceutical products. The AMA made a similar point about the importance of the speed of listing, indicating that, - while `the PBS is a good scheme in delivering low cost limited formulary medications to the Australian community, ... due to the low price that it is able to extract from international pharmaceutical firms, there is an understandable delay in getting new medications onto the market'.

Among the health professional groups, the Royal Australian College of General Practitioners (RACGP) representative interviewed noted that the PBS was successful in providing timely access and availability of drugs to treat most patients, but questioned the way in which some pharmaceuticals were added to and taken off the list. Among consumer groups, the Consumer Health Forum (CHF) shared the broad perspective that PBS provides effective and timely access to drugs for most people in most situations, but stressed the need for greater emphasis on the effects on consumers of both changes to PBS listing and administrative procedures.

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[11] Industry Commission, 1996, p 93.

On balance, ANAO concluded that the Scheme was widely regarded from diverse perspectives as being sound and as serving the Australian public well. This was not to deny that the principles behind the Scheme were also criticised by some observers. Also, as will be shown below, there were some strong criticisms of particular aspects of the PBS's operation.

3. EFFICIENCY AND TIME

3.1 Introduction

A key element of the audit was the question of whether DHFS' administrative processes for listing pharmaceutical products were efficient. The ANAO that considered the time taken to list products was an important indicator of efficiency.

Importance to different groups of stakeholders of time taken

From an overall public perspective, the first priority is to ensure that there is sufficient time for thorough evaluation of the benefits of each drug nominated for listing on the Schedule, and consideration of its value for money compared to that for other available treatments. This view has wide support among consumer and medical professional groups and sections of the industry.

On the other hand, various groups of stakeholders have reasons for shortening the duration of the listing process. Access to the latest medicines is important to consumers wishing to benefit from earlier availability of what may be an improved treatment. It is also important to health professionals to include the latest drugs in their choice of treatments for their patients.

Reducing the time to list drugs is a concern to the Government because of its objective of providing the Australian community with timely access to quality and safe products that are clinically sound and cost effective.

The shorter listing process is most important for the pharmaceutical industry to enable the early commencement of a return on its investment in research and development, including maximising the duration of patent time remaining after listing their products. This issue was identified by, and given a high profile in, the 1996 Industry Commission report[12].

Clarification of the roles of TGA and PBS

[12] Industry Commission, 1996, p 223.

ANAO found some confusion and complaint among industry and other stakeholders about the distinctive roles of TGA and PBS. Some clarification may be useful. Some industry representatives voiced the perception that there is overlap of functions and duplication between TGA and its Australian Drug Evaluation Committee (ADEC) on the one hand, and the PBS/PBAC processes on the other, particularly in respect of evaluation of clinical data.

ANAO notes that the roles and functions of TGA and PBS and their respective expert committees differ because the TGA and the PBS have different objectives. Therefore the work of their own staff and of their sub-committees serve different purposes, though sometimes necessitating analysis of similar data from sponsors.

TGA has a regulatory function to ensure that the safety, quality and efficacy of therapeutic goods available in Australia is at a standard equal to that of comparable countries. ADEC considers the efficacy and safety of particular drugs in absolute terms, while ADEC resolutions are used by the delegates in the TGA in approving drugs for registration for marketing.

The PBB has primarily a purchasing function, to ensure that the Pharmaceutical Benefits Schedule includes medicines which are clinically necessary and cost effective. PBAC is concerned with issues of comparative effectiveness and safety, usually compared to other drugs already listed on the PBS. PBAC's principal role is to determine value for money between competing manufacturers' drugs and under what conditions it might recommend listing for subsidy by the Government. The PBAC recommends to the Minister the drugs and medicinal preparations which should be made available as pharmaceutical benefits under the *National Health Act 1953*.

If the perceived overlap between the two committees relates to the same type of information being used for differing purposes, then there is no real overlap and the requirement is legitimate and necessary. From discussions between the ANAO, officers of the TGA and PBB and representatives of major pharmaceutical companies, ANAO concludes that there is little unnecessary duplication.

In recent years time for approval of new drugs has been reduced

When the listing of a drug is seen as a two stage process, comprising the registration for marketing by the TGA and the acceptance for subsidy by

the PBS, the total time for listing has reduced substantially over the past decade.

In 1991, at the request of the Commonwealth Government, Professor Peter Baume completed a major review of the Therapeutic Goods Administration (TGA)[13]. The review was in response to criticisms that TGA's evaluation of pharmaceutical products was too lengthy. Implementation of the Baume Report's recommendations saw the average time taken by TGA to evaluate new pharmaceutical products reduce from 702 working days in 1990 to an average in 1995 of 106 working days[14].

Besides these major reductions in the time required for the TGA process, the changes to the PBS process have had a less dramatic but still significant effect. Measures put in place in the PBS process to reduce the processing time, particularly for major submissions, include:

- an increase in the number of PBAC meetings from three to four each year. This has been accompanied by a reduction in the duration of the process cycle from between 170 and 185 working days before 1993 to between 145 and 160 working days for most process cycles since then;
- PBAC will now consider applications from sponsors once the TGA delegate gives a positive recommendation for marketing to ADEC (ie, before the TGA delegate's final approval), a potential saving of up to eight weeks; and
- the formalising of the 'cohort' system; a 'cohort' is a group of submissions arriving before the deadline for a particular PBAC meeting and, under its procedures, DHFS has guaranteed to consider all such applications at that PBAC meeting. PBB maintains that this practice avoids a backlog of applications.

Economy in the use of resources

Economy in the use of staffing and financial resources has a considerable bearing on efficiency. Since 1993, staffing levels in PBB have remained steady at between 60 and 65 people, until mid-1996 when staffing levels were reduced to between 55 and 60 people. Discussions with officers in

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[13] Baume Peter, *A Question of Balance: Report on the future of drug evaluation in Australia*, AGPS, Canberra, 1991.

[14] The Auditor-General ANAO Report No. 8 1996-97, *Drug Evaluation by the Therapeutic Goods Administration*, AGPS, Canberra, 1996.

the Branch and ANAO's observations suggested that resourcing levels were appropriate to the level of work required, except in the particularly busy times leading up to major events such as meetings of advisory committees. Indications from the number of applications, one of the major drivers of work in the Branch, suggested that the work load has increased somewhat over recent years, (see, for example, Table 3.1 column 5 below). ANAO concluded that the use of resources in PBB is economical.

3.2 Observations on the time taken to listing

This section notes the efficiency of the mechanics of the PBB's operation of the process, and then the wider issues of the operation of the process and the time taken to list.

PBB's own performance measures on time taken in the listing process

PBB measures its performance in administering the listing process in terms of the proportions of:

- applications received by the deadline which reach the next PBAC meeting;
- all positive PBAC recommendations which reach the next PBPA meeting; and
- all positive PBAC recommendations that are included in the earliest available Schedule, excepting delays caused by the sponsoring firm.

ANAO notes that applications approved at one stage are moved promptly to the next.

The need for and establishment of a listings database

At the commencement of the audit there were strong industry assertions about overly long processing times. The Department did not provide firm data to counter this view. Similarly, in the 1996 IC Review, some sponsors cited examples of applications taking a long time, but the Industry Commission had no way of determining whether these were exceptional cases or common occurrences because of the lack of data on the listing process. The Industry Commission commented that it had

'little independent information on which to base its assessment of the severity of the problems'.[15]

While individual sections of PBB monitor their parts in the listing process, the ANAO has had no indication that in the past the PBB had systematically compiled accurate information on the total time taken for individual applications to be listed, nor any measures of average duration of the PBS listing process for all applications.

To compensate for this lack of hard evidence the ANAO established a database of information (different parts provided by the different sections of PBB) to allow detailed examination of applications received since 1991. This database was essential to the analysis which follows. It was also relatively inexpensive to produce and simple to update. Such a database maintained by DHFS would be relatively low cost and in future be useful for providing more information to industry about the listing process and to counter claims by some parts of industry of the latter's excessive duration.

From analysis of those data, it was possible to distinguish between applications which were:

- accepted for listing after consideration in a single cycle of PBAC and PBPA meetings, prices agreed and the drugs listed within the minimum possible time. With four PBAC meetings per year this usually means 160 days or less to listing;
- accepted, but requiring two or more PBAC/PBPA cycles before being listed. These usually require a period of at least 210 working days and often more for listing; and
- not listed - either rejected at a PBAC meeting and still subject to reapplication and reconsideration, withdrawn by the sponsor, or recommended by PBAC but not listed because of non-agreement on price, or non-acceptance at the assay stage.

RECOMMENDATION 1

ANAO recommends that DHFS maintains an integrated database to monitor the progress of applications through the PBS listing process, and to provide relevant statistics on the efficiency of the listing process for management and for reporting to Parliament and major stakeholders.

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[15]Industry Commission, 1996.

DHFS Response:

Agreed. Work is under way to upgrade the computer tracking system for pharmaceutical benefit applications.

The analysis of time taken to listing

Figures 3.1 and 3.2 and Table 3.1 depict information extracted from the ANAO database concerning the time taken for the listing process. This information provides indicators of the efficiency of the process and whether efficiency is improving. The data are for the major applications as defined in Chapter 2.

Figure 3.1 shows the mean number of working days for approval of drugs submitted to each meeting from 1991 to 1996. The graph shows that there has been an overall decline in the time taken to list drugs. It also shows that there was a period in 1993-94 when the average time to list new products was temporarily longer than the preceding or following period - coinciding with the introduction of mandatory cost effectiveness analysis (see Chapter 4).

Two qualifications are needed about this general trend. Firstly, in 1991 the averages for the first two meetings were inflated by two applications which took an inordinately long time until approval (1273 and 945 days respectively). Secondly, for the most recent PBAC meetings, the average time for listing includes only applications approved up to the end of February 1997. Through 1997 and beyond, the average listing time for products first considered in 1996 (and to a lesser extent in earlier years) is likely to increase as previously rejected applications, currently in the pipeline, are decided upon, and hence their duration added into the calculation of the average.

On balance, it seems reasonable to conclude from Figure 3.1 that the average time to listing has decreased since 1993, though, bearing in mind the caveats in the previous paragraph, probably not to the extent the graph suggests.

Insert Figures 3.1 and 3.2

In Figure 3.2 the bar graphs show the proportion of applications for each PBAC meeting which were approved in a single PBAC/PBPA cycle. As noted above, for any particular application, the distinction between its processing in a single cycle only (160 working days or less), or its requiring a second or more cycles (usually 210 days or more) is crucial in determining the duration of time required before final approval for PBS listing. The line-graph joins the moving means - (the mean for each meeting is averaged with means for the two meetings before and two after to provide a smoother trend line).

Table 3.1 - Outcome of PBS applications by year of consideration by PBAC

Column 1	Column 2		Column 3		Column 4		Column 5	
Year (PBAC Meeting)	**Major applications listed in a single evaluation cycle**		**Major applications taking longer than a single cycle to listing**		**Major applications not listed**		**Total**	
	No.	**per cent**	**No.**	**per cent**	**No.**	**per cent**	**No.**	**per cent**
1991	18	60	9	30	3	10	30	100
1992	17	44	14	36	8	20	39	100
1993	13	34	14	37	11	29	38	100
1994	23	35	26	40	16	25	65	100
1995	17	36	12	26	18	38	47	100
1996	30	48	8	13	24	39	62	100
Total	**118**	**42**	**83**	**30**	**80**	**28**	**281**	**100**

Table 3.1 summarises the trends indicated in Figures 3.1 and 3.2 on an annual basis. Table 3.1 (column 2) indicates that the proportion of pharmaceutical products listed within a single cycle each year averaged 42 per cent over the period 1991-96. In 1991 there was a greater chance of

applications being approved in a single cycle than in subsequent years. From 1993 to 1995 the proportion recommended for listing after consideration in a single cycle remained comparatively low. The most likely explanation for this was the PBAC's introduction in 1993 of the mandatory requirement of the use of cost effectiveness studies with all major applications. This is discussed later in this report. In 1996 there was a sudden rise in first time listings to 48 per cent. While it may be premature to comment on the basis of the one year, the upsurge in 1996 may possibly reflect the growing integration of cost effectiveness analysis and associated amendments to the Guidelines[16] for the listing process.

Table 3.1 column 3 shows applications which are accepted by PBAC/PBPA as providing satisfactory value for money, but only after consideration at two or more cycles. These averaged 30 per cent of applications over the six years. The earlier years (1991-94) are more likely to be indicative of the proportions of applications needing more lengthy consideration, suggesting this might be typically between 30 and 40 percent of applications.

Table 3.1 column 4 indicates the proportion of applications not approved - some may have been withdrawn from consideration by the manufacturer (temporarily or permanently), some may be regarded as still `in the pipeline'. Since what happens to these applications depends on unknowns (whether sponsors will resubmit or not, and if so, whether PBAC will accept the applications) it is not possible to distinguish which drugs fall into which of these categories.

Comparison between the large proportion 'not approved' for the two most recent years (38 and 39 per cent) with the smaller proportions 'not approved' for the earlier years (ranging from 10 per cent in 1991 to 29 per cent in 1993) may give a broad indication of how many of those in the last two years may still be active applications. In other words, as PBS-industry negotiations on applications submitted in 1995 and 1996 continue, the 38 and 39 per cent of applications not so far listed will fall and the proportion listed after two or more meetings will increase.

Table 3.2 shows the duration of the listing process for applications considered at two or more cycles from 1991 to 1996.

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[16] Guidelines for the Pharmaceutical Industry, 1995.

Table 3.2 - Mean and median number of working days taken to approve major applications requiring consideration at two or more PBAC meetings; 1991-96

Measure	1991	1992	1993	1994	1995	1996	1991-96
Mean	519	329	438	355	285	221	359
Median	359	272	401	296	262	221	284

Table 3.2 provides the average listing time in the form of both the mean and the median. The mean and the median indicate a reduction, between 1991 and 1996, in the processing time for listing of applications requiring two or more meetings from respectively 519 to 221 working days (mean values) and from 359 to 221 working days (median values). The first caveat concerning both comparisons is that the 1996 mean and median statistics will rise with approvals of 1996 applications in subsequent years. This will apply also to a lesser extent to 1995 applications.

The significant difference between the 1991 and 1992 means and medians can be attributed to the inclusion in 1991 of a small number of applications which took an unusually lengthy period to gain PBS listing. These extreme cases exaggerate the mean but not the median and for this reason the median gives a better indication of the typical duration of the listing process for these applications in those years.

Table 3.2 also shows an increase in 1993 in the number of working days taken to approve applications considered at two or more PBAC meetings. As this report discusses later, this increase was associated with the introduction of the PBAC's requirement of companies to provide cost effectiveness data to support their applications.

A particularly important observation arises from Table 3.1 Column 5 on the total number of major applications per year. From 1991 onward there has been an increase in the number of major applications for PBS listing on a yearly basis from 30 in 1991 to 62 in 1996. This has

implications for PBB's work load and related resourcing needs, and for questions of efficiency of the process. To the extent that PBB has maintained or improved its efficiency over the years, it has done this in the face of a considerably enlarged workload. The PBB would not have been able to handle the workload without contracting out a considerable proportion of the evaluations to the University of Newcastle Evaluation Group. More detailed comments on the outsourcing arrangements are made in Chapters 4 and 6.

Comments on the observations

As described in Section 3.1, a cohort refers to a group of applications submitted in time for consideration at a particular meeting. Regarding the effectiveness of the PBAC's cohort system as a means of reducing delay in processing applications, the outcomes can be viewed in two differing ways. For applications which are approved in a single cycle, (nowadays taking usually 160 working days or less) the cohort system avoids a backlog. For applications needing consideration in two or more cycles the processing time is considerably longer (usually 210 working days or more). In these cases the comments of some sponsors that applications take a long time to be approved may be justified, though this delay can either result from the sponsor's slowness to resubmit or to the delays inherent in the listing process itself.

Care must be taken in trying to draw inferences from the proportions of approvals at particular stages and from any change over time. That is because, notwithstanding what was said earlier about time as a measure of efficiency, improvements in the efficiency of processes cannot be represented solely by simple reductions in the average number of working days necessary to schedule drugs, or by a very high proportion of pharmaceutical products gaining approval in a single cycle. The reason is that there will always be applications which the PBAC/PBPA must reject on clinical or value for the Commonwealth's money grounds. Also, as noted above, some delay is the direct responsibility of the sponsors themselves.

However, since a very large proportion of major applications is ultimately recommended for listing and subsidisation, it may be reasonable to expect improvements in efficiency to be reflected in an increase in the proportion of applications approved within the minimum time. In other words, a measure of improved efficiency would be if a higher proportion of major applications were approved the first time they were submitted by sponsors for PBS listing. The complementary

expectations of improved efficiency would be for the proportion of sponsored applications requiring more than one single cycle to be accepted for listing to decline, and for the median time of those requiring more than one cycle to decline.

Reduction in the duration of the process should not be an end in itself but be seen as a contribution to greater efficiency of the process without jeopardising its rigour. ANAO agrees that reducing the duration of the process is as much in the control of the sponsors as it is of DHFS. It is in DHFS' interests to make the application process more effective in getting suitable information for PBAC and PBPA consideration in order to reduce the number of re-submissions and hence improve the efficiency of its own use of time. Consequently, ANAO considers the aim to reduce the duration of the process, while inappropriate as a performance measure, should nonetheless be adopted as a significant goal by DHFS.

The ANAO concludes there are opportunities for reducing the time taken to list drugs on the PBS within the present arrangement.

Recommendation 2

ANAO recommends that DHFS explores ways to reduce the average time taken to list drugs on the PBS insofar as this is consistent with rigorous evaluation and value for money, through avenues such as:

- avoiding delays to correct relatively minor inadequacies in sponsors' applications for (PBS) listing;
- increasing the proportion of applications accepted for listing in the first cycle of evaluation;
- more effectively using IT resources to support operation of the listing process; and
- reducing the time taken to produce the PBS Schedule.

DHFS Response:

Agreed with qualifications. Minor reapplications are already accepted after the cut-off date for major applications. As recognised in the ANAO report, the rigour of recommendations to ensure consistency with legislative obligations is paramount and the proportion of applications accepted for listing in the first cycle is not necessarily consistent with this responsibility. However, an objective of the PBS is to provide timely access to new benefits and the Department will continue to pursue this

objective. The Department is exploring ways to supply the Schedule in an electronic format.

The above recommendation could be implemented by considering, among other means, suggestions in the following sections of this report.

3.3 Possible improvements

This section discusses only improvements in the efficiency of the listing process which might be gained by reducing the time taken to process applications without major alterations to the structure of the present listing process.

The suggestions have all been raised by or with industry and discussed with DHFS which was already taking action.

a. Avoiding a long delay to correct a minor inadequacy in an application

A common industry complaint was that where a relatively minor inadequacy in an application results in PBAC rejection, there is a disproportionately long delay before the PBAC reconsiders the submission. This is because the PBB procedures make it difficult for early reconsideration. In turn, this arises because the cut-off time for the next PBAC meeting follows closely on the one at which the submission has been rejected - usually within two weeks and sometimes less. Where a submission is rejected at one meeting it is highly unlikely that the resubmission could be prepared in time for the next meeting, but rather would make the following one, adding up to six months to the listing process.

Solutions proposed by industry included provision of a later closing date for the next meeting for applications needing only relatively minor amendments or additions. Alternatively, a sub-group of the PBAC could consider (and if appropriate approve) minor changes to a submission soon after the particular PBAC meeting and allow the application to proceed to PBPA.

Another suggestion was to increase the number of meetings of PBAC from four to six per year. This would mean that applications rejected at one meeting could not expect to be resubmitted at the next meeting, but the time to wait for the next but one would be considerably shorter - four months rather than six. ANAO notes, however, that an arrangement with six meetings per year would require overlapping schedules between meetings and extra workload in administering the listing

process. These complications could detract from the current efficiency of the process and consequently ANAO does not recommend its adoption.

b. Increasing the proportion of applications approved at a single cycle

The second area of possible improved efficiency is through an increase in the proportion of applications approved on the first occasion they are submitted by sponsors to the PBAC. ANAO considers this the area offering some limited potential for improved efficiency both in the short and long terms. This improvement would require PBB to provide more assistance to pharmaceutical companies to submit better quality applications. It would also require greater understanding and appreciation of the DHFS-PBAC Guidelines, which could be achieved through their improved promotion and increased PBB - PBAC consultation with and provision of information to industry. This is not to infer that the main responsibility in this respect rests with DHFS. The achievement of any lessening of the duration process would depend primarily on industry responses to DHFS' overtures. These matters are discussed in Chapter 4.

c. Special attention to effective use of IT resources

DHFS Internal Audit in its report, *Internal Audit of Evaluation, Registration and Listing of Drugs on the Pharmaceutical Benefits Schedule-(November 1995),* recommended that the PBB develop strategies for more effective application of contemporary IT to its information management processes. Internal Audit considered that this would achieve efficiencies from:

- faster input to tracking and data management systems and initial checking and validation of submissions;
- faster transfer and more consistent management of information within the PBB; and
- improved evaluation of submissions by having consistent data formats and analysis facilities.

Industry representatives observed that the listing processes - from submission of applications to the publication of the Schedule and the production and distribution of the Schedule - could become more efficient and be completed in a shorter time frame if there were a greater use of information technology.

Based on observation and discussion during the audit, the ANAO supports the findings of DHSF Internal Audit and considers that there are two main areas where the use of information technology can improve the efficient operations of the PBB:

- acceptance of submissions in an electronic format, for example using E-mail or eventually even the Internet; and
- in the production of the PBS Schedule.

d. Reduction in the time to produce the PBS Schedule

The Schedule of Pharmaceutical Benefits (The Schedule) is a volume of between 300 and 350 pages, including detailed lists of the pharmaceutical products and the prices of which the Government has agreed to subsidise[17].

The Schedule identifies product names, their restrictions, authorisations, manner of administration and form (eg, tablet or cream). Medical doctors in private practice, dental practitioners and approved pharmacies receive complimentary copies of the Schedule by mail.

Currently, as shown in Figure 2.1, ten weeks are taken up with editing, printing and distributing the Schedule. The time taken does appear disproportionate compared to other parts of the listing process, which in total takes 32 weeks. However, while there may be opportunities for very minor savings of time, ANAO did not find, scope for great savings in time within the current system.

In a longer-term perspective, several industry representatives and professional bodies saw the opportunity for the electronic production and distribution of the Schedule. The PBB should consider the future provision of the Schedule to pharmacists and general practitioners (GPs) in an electronic format.

Computerised production and distribution of the Schedule will depend particularly on having the great majority of users computer-literate and adequately equipped. It is essential to the operation of the system that all GPs and pharmacies have access to the revised Schedule on the appointed day. While almost all pharmacies use computers, it is

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[17] *Schedule of Pharmaceutical Benefits, For Approved Pharmacists and Medical Practitioners*, Commonwealth Department of Health and Family Services, AGP.S, Canberra, 1996.

estimated by the PBB and other industry commentators that currently the proportion of GP practices using computers is far smaller. Informed estimates suggest only about 25 per cent of GPs use computers for financial records and accounts, and only five per cent use a computer to assist with their clinical practice, such as in maintaining patient records.

Both the Australian Medical Association and the Royal Australian College of General Practitioners have recognised that the low level of computerisation of GPs' offices is a barrier to electronic distribution of the Schedule. However, the potential benefits are considerable and this makes electronic publication a desirable medium term objective.

The ANAO noted that the PBB had employed a consultant to explore the feasibility of electronic prescribing and to develop functional specifications for computer systems for use by GPs. The first of the consultant's reports, completed in early 1996, had a number of key findings, including that implementation of medication management/electronic prescribing in Australian office-based practice could achieve major savings in the national health budget but that significant incentives might be required to encourage medical practitioners to use computers more in office management and in medical practice. The second report produced in 1997 has supplied functional specifications for appropriate software systems.

4. SELECTION OF DRUGS FOR PBS LISTING

4.1 Introduction

Outline of the audit of performance in the listing process

This chapter reviews the introduction of the evidence based approach to selection of drugs for PBS listing and the use made of economic analysis in the process.

Three main factors influence the benefits to be gained from use of an evidence based approach in the selection process. These are:

- the adequacy of the Guidelines provided by DHFS to would-be applicants for listing their pharmaceutical products;
- the quality of sponsors' applications in response to the Guidelines; and
- the extent and way in which the advisory and decision-making bodies use the information in their own decision processes.

These factors involve complex technical questions. The ANAO used a consultancy to investigate and report on these issues. A digest of the issues and main conclusions from the consultant is provided at Appendix 1.

In conclusion, the chapter covers two important issues frequently raised by industry; firstly, the scope of the evidence considered in the listing process, and in particular the consideration given to potential 'non-pharmaceutical' offsets associated with the listing of new drugs; and secondly, the transparency of the listing or scheduling process to the pharmaceutical industry.

4.2 Evidence based approach to selection of drugs for PBS subsidy

In order to guide Government in the selection of which pharmaceutical products it will purchase through the PBS, sponsors are now required to provide evidence of the benefits and costs to the Government from the purchase, utilising well conducted studies with thorough and rigorous clinical trials and economic analysis. This is termed the evidence based approach.

The alternative to this approach is the more traditional opinion-based approach relying on expert opinion plus observations about trends

current in contemporary practice. This was the approach used in DHFS' considerations in the past.

Australia was one of the first countries to introduce an evidence based approach in a government's selection of drugs for purchase and subsidisation. In 1991, the optional use of cost effectiveness data by sponsors, as part of an evidence based approach, was introduced by the Commonwealth Government. This became mandatory in 1993 for companies sponsoring inclusion of drugs on the PBS.

The evidence based approach was introduced into the PBS because it was thought likely to provide better assistance to PBAC decision making, and less likely to be accompanied by lobbying as sometimes occurred with the opinion based approach.

Cost-effectiveness studies in the PBS context are used to demonstrate whether a drug proposed for listing provides greater benefit than the alternative therapy to which it is being compared. For example, a drug may have a higher price but achieve the desired clinical outcome in a higher proportion of patients than the alternative. The summary measure of cost effectiveness analysis is the incremental cost per additional unit of outcome achieved.

ANAO found that industry understanding of the Government's initiative in this area varied considerably. Some industry commentators still regarded the evidence based approach to be something unique to Australia and necessitating research and justification in submissions which were not needed in other countries. A growing majority of company representatives appears to be more aware that Australian practice is part of a strongly emerging trend in Europe and North America for governments and health authorities to use economic analysis in selection of drugs for purchase or subsidisation. The ever growing cost of pharmaceutical products and the related cost of poor purchasing decisions underlies this trend.

Similarly, regarding the acceptability by industry of the form in which economic analysis is used in the PBS listing process, industry has a wide variety of views but on balance tended to be positive. This differs from the impression gained from the 1996 Industry Commission report. This noted that while many of their industry informants 'accepted that economic analysis has a role to play and supported the use of cost effectiveness in the listing process, . . many were concerned that cost

effectiveness requirements were being used inappropriately'[18]. The difference in the impressions gained by IC and ANAO is significant and could have arisen from differing methods used to elicit industry views.[19]

Twelve of the 16 major companies which the ANAO interviewed had frequent experience of the listing process. The other four companies were concerned mainly with generic drugs or a particular niche in the market, and their experience with preparing applications using rigorous economic analysis was limited. Based on the views expressed by CEOs and/or other leading executives in these 12 companies, the companies can be placed into one of three groups in regard to their attitude to the evidence based approach:

- three firms had negative views and were essentially averse to working in the PBS system. One CEO stated that 'the PBS in its present form has outlived its usefulness and needs major surgery'. This perspective holds that PBAC's rigid data requirements created unique demands for pharmaceutical companies, and that, while Australia was a world leader in the use of cost-effectiveness studies, the benefits from leadership were questionable, and possibly negative;
- six firms were positive about the (evidence based) rationale behind the listing process, but with varied and sometimes strong reservations about the operation of the Guidelines and cost effectiveness analysis requirements; and
- three firms were positive about the PBS' rationale, and had no or only minor reservations about its operation.

In this context several observations are relevant:

- the Chairman of PBAC saw an improvement in the overall quality of submissions since the introduction of the cost effectiveness guidelines and, in his view, this was making PBAC consideration more systematic and easier;
- there seemed to be a relationship between the more positive attitude by some companies to the evidence based approach (see above) and their experience and success in having applications approved. For example, on the basis of information in the ANAO database,

[18] Industry Commission, 1996, p247

[19] The IC took written submissions and held public meetings with a range of manufacturers. ANAO held discussions with CEOs and executives of a sample of the industry, from 16 major companies. It may have been that in the more informal discussions with ANAO the same people may have been able to canvass a wider range of views.

companies were ranked on a 'success scale' (combining overall success in having applications accepted as opposed to being rejected and having applications for listing approved expeditiously in a single cycle). It was noted that those companies with the more positive approach tended to be in the top third on the 'success scale' whilst those with the negative approach tended to be in the bottom third of the scale. However, it was not possible to determine whether a company's views on PBS affected its success with cost-effectiveness studies or vice versa; and

- the more positive companies characterised themselves in discussion with ANAO as viewing DHFS (through the PBS) primarily as a customer like any other (not as primarily an arm of government), and as having invested in the recruitment and employment of a significant number of health economists to cater to the information needs of this customer.
- the industry views, characterised in the three groups noted above, may represent a continuum in the acceptance of the innovation of the evidence based approach from initial negativity to acceptance into the mainstream of the process.

4.3 The Guidelines

DHFS uses the Guidelines to provide assistance to sponsors in their preparation of applications. There have been two versions of the Guidelines to date. They are the 1992 version, which is seen in retrospect by industry as simple and flexible, and the current 1995 replacement version which is more detailed and technical. A further revision is planned by DHFS for 1999.

The Guidelines provide the format in which an applicant for listing of a drug is asked to submit information in support of its application. This includes a description of the drug and its uses, the details of its clinical performance compared to the therapy most likely to be displaced, an extrapolation of the clinical effect of the drug on patient symptoms into patient relevant outcomes, and an analysis of the effect of listing the drug on the PBS and on public sector health budgets.

The Guidelines provide a framework for rigorous analysis of the clinical benefits and possible limitations of the drug and for consideration of value for money issues. There is extensive guidance on the preferred basis of comparison with the comparator drug.

The content, presentation and application of the 1995 Guidelines, especially in respect of the requirements for cost effectiveness studies, are contentious with some sections of industry. The differences of opinion are not about the consistency of the Guidelines with Government policy or legislation, rather the mode of operation and application of the cost effectiveness analysis as part of the process. As was highlighted earlier, this was one of the main areas where some strongly negative reactions from industry arose - with the strength of the disquiet varying greatly between companies. DHFS maintains that the 1995 version was prepared in response to industry requests for more DHFS-PBAC direction than provided by the earlier 1992 Guidelines. Even though there was considerable industry input, the revised 1995 Guidelines are now seen by sections of industry as prescriptive, complex, onerous and demanding.

The ANAO consultancy concluded that, overall, the Guidelines are rigorous and potentially useful to companies drafting submissions. The consultants noted that the Guidelines `provide a highly succinct handbook on the combining of clinical and economic analysis which must command respect'. In the Guidelines, the consultants see PBAC as identifying the standards it is seeking in company submissions and providing a high level of assistance to companies in meeting those standards.

Two groups of issues deserve audit comment: firstly, the content, formatting and presentational requirements in the Guidelines and, secondly and perhaps more importantly in the longer term, the technical development of the economic analysis required by the Guidelines and ultimately the rationale underpinning the decision making process.

Clarity and user friendliness

Some industry representatives complained that the Guideline were not user friendly, were lacking in clarity, too onerous, too prescriptive, unnecessarily complex and confusing. ANAO raised these concerns with DHFS and PBAC. Regarding the clarity issue, they noted that the 1995 Guidelines were prepared under some time pressure and that, ideally, industry consultation might have been more extensive.

The consultants noted that while some industry representatives saw the information requirements for drug approval for Australia as more demanding than in much larger overseas markets, others took a different view. One leading company noted that the information sought by PBAC

is soundly based in economic analysis, and is of a type increasingly demanded by other major customers overseas.

ANAO notes that a further revision is currently under way and is aware that PBB is conscious of the need for consultation with industry and other stakeholders and of the importance of achieving clarity of presentation. Given the variety and complexity of the technical issues involved, the current Guidelines, while not easy to read, are comprehensive and readily useable by people with adequate expertise. In addition, reference to the growing literature on this branch of economics will assist would-be applicants to understand the underlying logic. With greater familiarity through repeated use, several health economists with industry expressed positive opinions on the utility of the Guidelines.

Notwithstanding those issues, the regularity of industry criticisms of the cost effectiveness Guidelines pointed to the need for DHFS-PBAC to focus on exploring possible improvements within the existing policy framework.

Technical developments in economic analysis

ANAO notes that, in the PBS listing process, the overall standard of analysis by the Department's advisory committees was very high compared to many other Commonwealth programs.

Nonetheless, there were some important advances of a technical nature necessary to make the analytical process more comprehensive and reliable. DHFS has been working on these issues, especially through the PES and the Economic Sub-committee (ESC).

The rationales supporting the following suggestions are discussed in the digest of the consultants' report at Appendix 1. Foremost in the suggestions for improvement are:

- DHFS should consider the use of more uniform outcome measures of the consequences of adding particular drugs to the PBS Schedule which would be capable of supporting a broader generality of conclusions across therapeutic fields;
- PBAC should consider making its measures of technical efficiency more explicit or consider ranking drugs in order of relative efficiency, either of which might assist decision making and choice within particular drug groups;

- sponsors could be encouraged to provide cost benefit analyses which permit comparison of benefits across different groups of drugs. This would have considerable advantage over cost effectiveness studies which are more limited in this respect;
- the more uniform and versatile outcome measures referred to above could therefore be obtained by expanding the scope of analyses in sponsors' applications. This could be achieved by expanding the analysis into measuring allocative efficiency across a wide range of applications rather than the more limited technical efficiency which is possible at present (see Appendix 1 for further explanation).
- a more sophisticated use of sensitivity analysis by companies, if properly designed, would increase the transparency of possible defects in cost effectiveness analyses;
- the importance of a decision process which enables the reconciliation of qualitative evidence on claimed intangible benefits from use of a drug (such as equity benefits), with quantitative data needs to be acknowledged by the Department, and encouraged in the next edition of the Guidelines;
- the value of making more explicit the threshold values for listing of applications.

Recommendation 3

To improve the quality of the economic analysis required of sponsors in submissions for PBS listing, the ANAO recommends that, in its revision of the Guidelines for the Pharmaceutical Industry on Preparation of Submissions to the PBAC, DHFS consider incorporating into the Guidelines a number of technical developments, involving among other things:

- the more discriminating use of sensitivity analysis;
- better articulation and integration of qualitative assessments of intangible benefits (eg, equity considerations) with quantifiable measures;
- the more frequent use of cost benefit analysis; and
- the development of more uniform outcome measures to allow comparison of the value for money to the health system of drugs addressing different indications or medical conditions.

DHFS Response:

Agreed. The Department and the PBAC are committed to continuously improving the PBAC Guidelines. The items suggested for incorporation in the PBAC guidelines by the ANAO are all rather controversial issues in the areas of health economics. The Department currently is finalising briefs to invite tenders to carry out literature reviews of some of these issues with the aim of providing discussion papers for the pharmaceutical industry and other stakeholders in a workshop planned in 1998. The feasibility of incorporating the suggestions by the ANAO will be reconsidered following these consultations.

4.4 The quality of applications from industry using the Guidelines

ANAO notes that while the quality of the applications received by PBB from the pharmaceutical industry is variable, the quality has improved as industry has become more used to the process and more familiar with the Guidelines. This view is based on opinions expressed by leading members of the advisory committees, by senior Departmental officials, and by industry representatives themselves. It is a view supported by the ANAO's consultants' analysis of a sample of applications submitted in recent years.

The ANAO also noted that while the Department and its advisory committees encouraged high sponsor standards of data presentation, analytical rigour and a high overall quality of information, these bodies administered the standards in a sensible and flexible way.

Through a detailed analysis of a sample of applications, the ANAO's consultants concluded that `the substantial number of defects recognised . . . suggests that there is still some way to go before best practice in the development of cost effectiveness information by proposing companies is achieved'. The consultants considered this may reflect the steep learning curve for companies adapting to the new information requirements, and that variations in the quality of submissions suggested that there was scope for under performers to catch up with best practice.

Recommendation 4

ANAO recommends that DHFS has in place a procedure to record the strengths and weaknesses of the use of cost effectiveness analysis in individual sponsor applications in order to guide its advice to industry.

DHFS Response:

Agreed. This aspect will be included in the upgrade of the tracking system referred to in the response to Recommendation 1.

On going assistance

More DHFS-PBAC assistance to industry was warranted to encourage better use of the current Guidelines, particularly in regard to the cost effectiveness provisions and especially for firms with relatively little experience of the latest developments in the Guidelines. The extra effort required of PBB would be an investment in that it should show a return in smoother and more efficient processing once applications are received.

Recommendation 5

ANAO recommends that DHFS increases its promotional efforts and guidance to less experienced sponsors of new drugs in the PBS listing process to allow these sponsors to more quickly comply with the Guidelines and provide information of high quality.

DHFS Response:

Agreed. The Department participates in many industry meetings and conferences intended to explain the policy and requirements for economic analysis. The University of Newcastle is contracted to provide educational workshops on aspects of the Guidelines to the industry and, in conjunction with the Pharmaceutical Evaluation Section (PES), is planning two workshops next year to assist the industry in this aspect.

Detailed pre-submission consultation

Some major companies proposed to ANAO that there be more formal discussions between sponsors and DHFS in regard to sponsors' listing proposals. The Department informed ANAO that it is willing to discuss sponsors' applications before they are submitted, but that not all would-be applicants take advantage of this possibility. ANAO encourages DHFS to provide opportunities for sponsors to make presentations to the Department in the later stages of development of applications.

Recommendation 6

ANAO recommends that DHFS considers initiating more effective face-to-face consultation with companies following initial assessment of their more complex submissions, in order to:

- provide companies with more knowledge of the listing process; and

- clarify as many issues and data requirements as possible before they are provided to the Department's advisory committees.

DHFS Response:

Agreed. DHFS has an open door policy for assisting pharmaceutical companies prior to making PBS submissions and after they have been considered by the PBAC. Substantially more resources will be required to have formal meetings during the evaluation period after applications are made due to the tight time frames and workload at this period and to provide time for sponsors to respond to issues raised. Minor matters in applications are already dealt with through contact with applicants, and applicants have the opportunity to provide written responses to issues raised in the evaluation and review by Department.

Future revision of the Guidelines

In preparing the next revision of the Guidelines, the Department could give even greater attention to clarity, with better written presentation and with even more industry involvement than has occurred to date.

ANAO concluded that there could be benefit for DHFS to give higher priority to revision of the Guidelines, particularly considering the perceived need by industry for greater clarity, and more especially to introduce various technical changes in the preferred types of economic analysis discussed in section 4.3 above and in the digest of the consultants' report (Appendix 1). The Department could give higher priority to revision of the Guidelines by contracting out more of the evaluation of applications for listing to free up its specialised staff, or by contracting out some of the development work itself.

In addition, ANAO notes that the current plan is to have a major revision of the Guidelines in 1999. Given that valuable developments in methodology (both major and minor) are being discussed and accepted continually, ANAO considers that there are advantages in these being introduced as they arise rather than holding developments back until a major revision is finalised.

Recommendation 7

ANAO recommends that DHFS gives greater priority to the revision of the Guidelines for the Pharmaceutical Industry on Preparation of Submissions to the PBAC so that technical improvements currently being considered can be quickly refined and integrated into the selection

process and that these improvements be added to the Guidelines progressively rather than waiting until 1999 for a new edition.

DHFS Response:

Agreed with qualifications. DHFS in consultation with the APMA monitors and makes minor adjustments to the Guidelines. The Department will consider ways of progressively incorporating more major changes, as outlined in the response to Recommendation 3. The priority assigned to the revision of the Guidelines depends on resources remaining after meeting essential functions to maintain the listing process.

4.5 Decision makers' use of economic analysis

Some industry concerns with the Guidelines and their implementation

There were a number of issues raised by industry which, after further enquiry and research, ANAO concluded did not constitute a major problem in the listing process, although they may have appeared so in the past.

Some industry representatives complained that on occasions the agreed comparator against which a new drug should be compared in the PBS application was unilaterally changed by DHFS. A distinction should be made between disagreement (between industry and PBAC) over the initial selection of a comparator, and late change to an agreed comparator. DHFS documented 31 occasions on which it and industry disagreed about the suitability of comparators included in submissions. However, in the majority of cases PBB had not been consulted earlier, and in several cases where it had, the sponsor chose an alternative. Changes to an agreed comparator, however, were very few.

Other industry representatives noted there were times when they were asked to provide information to DHFS to assist on a decision on PBS listing when similar information had already been provided to TGA and accepted in the TGA context. This issue was examined in Chapter 3 in relation to the roles of TGA and PBS. ANAO concluded that while similar information may be required for TGA's and PBS's processes, it is usually for significantly different purposes and therefore the requests have been legitimate.

Nevertheless, instances which led to the above criticisms could have had very serious consequences for sponsors when or if they occurred.

Because of their infrequency, they were not major impediments to the efficacy of the selection process as a whole. The problem seemed to be one of industry perceptions that these cases were more frequent. DHFS may need to devote some time to redressing these mis-perceptions.

Some sections of industry currently considered the Guidelines and cost effectiveness as simply cost containment measures. This perspective may also reflect industry expectations and perceptions of the listing process. By way of contrast, some sponsors operate within the current system comfortably and successfully. This raised the question of why all companies could not do this, and pointed to the importance of developing the right perceptions and encouraging positive approaches among industry executives.

A particularly persuasive comment from some industry representatives was that cost effectiveness was more an art than a science, inferring that the discriminating power and precision of economic methods could be overestimated. In further developing the Guidelines, a major benefit from additional DHFS consultation with industry should be an increase in the latter's confidence in the selection process.

4.6 'Non-pharmaceutical' savings in the economic analysis

An item raised frequently with ANAO by industry was the issue of the importance accorded in the cost effectiveness analysis to those savings which might occur in non-pharmaceutical areas as a result of PBS listing of a particular drug. A frequently cited example was the claim that use of a pharmaceutical product can reduce the need for users to be admitted to hospital. Increased Commonwealth expenditure through a higher subsidy for such a pharmaceutical product would be offset by savings to hospitals as fewer patients suffering from the relevant health problem are admitted, or, if admitted, they are admitted for less time. According to this line of argument, this in some cases could represent considerable savings to the Commonwealth which could justify premium prices for the products.

The views expressed by industry and the rejoinders from DHFS suggest that there is considerable misunderstanding amongst industry of the status and treatment of such potential savings in the evaluation part of the listing process. DHFS could find considerable advantage in reducing industry's misperceptions.

Some industry commentators protested that such non-pharmaceutical offsets should be allowed. This is in some measure an indication of industry's lack of understanding of the Guidelines since these state that

financial information on such direct benefits for the health system (as well as for users) can be used in support of an application for listing.

More often, industry representatives were aware of the admissibility of this evidence, but gave the opinion that such data were given much less weight in DHFS-PBAC deliberations than clinical evidence in direct head-to-head trials showing advantages over other pharmaceuticals.

Whatever the case, a common view among stakeholders was that DHFS and its advisory committees were seen as making PBS expenditure decisions without sufficient regard for the benefits of that expenditure for the rest of the nation's investment in health care. ANAO is mindful that PBS has a higher level of rigour applied to the evaluation of drugs than most, if not all, other areas of health purchasing. Evidence to confirm the potential likelihood of claimed savings may be hard to find because of the inadequacy of costing information in these other areas of health spending. Similarly, it is difficult to follow-up and both confirm that the savings do in fact occur and to realise them.

The ANAO consultant's report noted that in terms of costs and benefits, there are strict limits to the extent to which non-pharmaceutical offsets can be seen as legitimate offsets . For example, in the case of potential savings from successful treatment of an individual's illness by pharmaceutical products rather than by hospitalisation, direct financial savings from use of that bed would be few because the bed would not be unoccupied or 'abolished' but occupied by the next person on the waiting list. In this case the benefit would be the shortening of the waiting time of a person removed from the waiting list. Since these issues do cause some controversy, there could be benefit in the Guidelines clarifying the precise nature of the offsetting benefits which may be reasonably considered.

Recommendation 8

ANAO recommends that DHFS clarifies, in the Guidelines for the Pharmaceutical Industry on Preparation of Submissions to the PBAC, the context and the extent to which sponsors, in their submissions to PBAC, can use data on potential cost savings elsewhere in the health system, such as in hospitals, through use of pharmaceutical products.

DHFS Response:

Agreed. The Guidelines, together with the associated Manual of Resource Items and their Associated Costs, clearly specify that the full

average cost should be used to estimate cost savings expected to occur elsewhere in the health system. The revision process of the Guidelines will consider the ANAO's suggestion that this over-estimates the true cost savings.

4.7 Transparency of decision-making

Industry representatives raised with both the ANAO and the Industry Commission their dissatisfaction with the relative lack of transparency of DHFS - PBAC decisions, and the inadequate level of information from DHFS-PBAC on their responses to sponsors' submissions.

In recent years there have been several developments to enhance the openness of the PBS process:

- since July 1993, PBAC members have met APMA representatives annually to discuss issues of concern to either party ;
- APMA nominees have been appointed to the PBAC Drug Utilisation Sub-Committee since 1994 and to the Economic Sub-Committee since 1994. The Drug Utilisation Sub-Committee monitors the patterns and trends of drug use and makes that data available to Australian and international consumers, research groups and interested bodies. To avoid conflicts of interest, APMA, rather than individual firms, nominates representatives to these Sub-Committees; and
- from 1994, the Department has provided sponsors with a summary of its evaluation of their submissions prior to PBAC's meeting. This information offers sponsors the opportunity to provide a rejoinder for PBAC consideration. In addition, since the beginning of 1994, following the PBAC meeting where an application is rejected, sponsors receive a summary of the reasons and relevant parts of the Committee's minutes.

ANAO noted that DHFS has maintained detailed correspondence with the pharmaceutical industry on drug applications , and has an open door policy to industry representatives wishing to discuss issues concerning their applications for PBS listing.

APMA acknowledged that DHFS-PBAC provided more information now than in the past, but suggested further enhancements. These were that the PBAC-APMA meeting might be more frequent than once per year, and that information on Departmental and ESC advice to PBAC be fuller, with more time for industry to respond. ANAO raises these matters for the Department and PBAC to consider further.

5. PBS AS A PURCHASING PROGRAM

5.1 Introduction

Section 85 of the National Health Act states that pharmaceutical `benefits shall be provided by the Commonwealth' in accordance with the Act. The role of providing drugs and medicinal preparations places on the Commonwealth the complementary role of ensuring that the drugs are available, and of being a major player in the purchasing process.

This chapter reviews the implications for the listing process of the Commonwealth's purchasing role, including:

- the PBS as a purchasing program;
- the PBS (PBAC/PBPA) decision-making;
- reviews - for price revision, and for improved decision-making;
- PBS and promotion of pharmaceutical benefits to GPs and to the public;
- some cost containment issues.

5.2 The PBS as a purchasing program

In the transaction between the producer of pharmaceutical goods provided under the PBS and the consumer (and over-the-pharmacy-counter 'purchaser'), the Government has an essential intermediary role of provider of pharmaceutical benefits. The distinction of which is the purchaser, the consumer or the Government, is not clear cut.

In the Australian market context, the Commonwealth is seen as the major purchaser to the extent that it has a monopsony position. A monopsony exists where there is a dominant purchaser in a market with multiple sellers. In the actual purchasing transaction, it is the consumer who makes the purchase over the pharmacy counter while the Government has a crucial facilitating role through its provision of the subsidy. This subsidy element is the major part of the purchase cost in that, of the drugs purchased under the PBS, the Government bears approximately 83 percent of the total cost ($2.538 billion in 1996-97) and the consumer about 17 percent ($530 million in 1996-97). Also, the Government has a role in determining the price through its negotiation

with the supplier. This is reflected by the fact that the consumer's payment is essentially fixed by his or her status (whether concession holder or not) and it is the Government's payment which varies according to the price which it has agreed with the supplier.

Thus while the precise definition of the 'purchaser' is not entirely clear, for practical purposes it is the Department which has most of the characteristics of the purchasing role and is best seen as at least the proxy or virtual purchaser. It is the principal player on the purchasing side of the market equation and the one most able to exercise direct influence and protect consumers' interests. In addition, it is directly responsible to the Government and Parliament for administering the overall cost of the program through its position under the National Health Act as provider of pharmaceutical benefits.

As mentioned earlier, through the PBS the Government subsidises each year on behalf of the public approximately $2.5 billion of pharmaceutical products. The cost of the program has grown at between eight and thirteen per cent in real terms in recent years. resulting in a doubling of government outlays in little more than six years. This is a major budgetary concern.

An examination of all the factors affecting growth in Commonwealth outlays under the program was beyond the scope of this audit. It did, however, focus on the listing process as one important factor.

The listing process, as a pharmaceuticals purchasing process, is integral to this expenditure growth. Although PBS operates by means of providing subsidies to transactions between the patient and the pharmacist, it is essentially a purchasing program on a major scale. It is a special instance of the purchaser-provider model.

ANAO has noted changes in how DHFS describes its responsibilities as a purchaser of drugs:

- in 1995-96 the objective of the Pharmaceutical Benefits Program was ' to provide access to necessary therapeutic substances at the lowest cost to Government and consumers consistent with reliable supply';[20]
- in 1996-97 the goals included ensuring that the PBS schedule 'includes all necessary medicines reflecting modern and appropriate treatment',

[20] *Portfolio Budget Statements 1995-96, Health and Family Services Portfolio*, AGPS, Canberra, 1995, p.83.

and achieving efficiency in the PBS 'including the listing process and the containment of cost of the Scheme'[21];

- in 1997-98 the objective of the program was stated as 'to provide timely, reliable and affordable access for the Australian community to necessary and cost effective medicines'[22];

- the requirement for acquiring the benefits at the lowest cost to government was dropped from the program's objective in 1997-98. However, it is retained at the second level of goals or priority outcomes.

- in 1997-98 the goal (styled 'priority outcome') was changed to ensuring the schedule of PBS 'includes only medicines which are clinically necessary and cost-effective' and achieving efficiency in the PBS, including in the 'listing the process and appropriate management of the cost of the scheme'[23].

Rather than have a Budget limit predetermined as with most appropriations, PBS has a Special Appropriation and is demand driven. In such a case, the Budget outcome each year depends on the level of demand by consumers for the goods provided by the program. This makes some conventional purchasing and budget management operations difficult to apply and operate.

In order to assess the effectiveness of the PBS as a purchasing program, ANAO used as a benchmark the Department of Administrative Services Interim Procurement Guidelines (referred to below as the Procurement Guidelines)[24]. ANAO observed that the Procurement Guidelines were not designed for a demand driven program of this kind. Nonetheless, the Procurement Guidelines, in providing advice to agencies on how to purchase goods and services consistent with Government policies, set standards for Government procurement across all programs.

Table 5.1 Comparison of DAS procurement cycle and DHFS pharmaceutical purchasing

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[21] *Portfolio Budget Statements 1996-97*, p.119.

[22] *Portfolio Budget Statements 1997-98* p.131.

[23] *Portfolio Budget Statements 1997-98*, p.131.

[24] *Interim Commonwealth Procurement Guidelines*, Department of Administrative Services, AGPS, Canberra, 1997.

	Stages of DAS procurement cycle	PBS listing process counterpart
1	identifying and justifying agency needs and then assigning priorities	reacts to industry initiative - no ranking for specific drug needs
2	defining requirements and developing the business case for major procurements	reacts to industry proposals - no preliminary investigation of overall pharmaceutical needs
3	researching and developing business plans and strategies	reacts to industry proposals - no advance plan of purchases
4	qualifying competent suppliers	affected through TGA process
5	preparing specifications	provided to would-be sponsors in the form of PBAC guidelines
6	seeking offers in the market place	market offers formalised through PBAC guidelines
7	evaluating offers	extensive evaluation by PES, ESC and PBAC
8	negotiation	parameters set by PBPSA on PBAC advice; negotiation by PBB
9	contracting with suppliers	listing on the PBS Schedule
10	debriefing suppliers and handling complaints	PBAC and PBB consultation with industry
11	managing contracts to ensure performance	HIC processes with selective annual price reviews by PBPA
12	evaluating outcomes	DUSC monitors drug usage but not systematically; limited monitoring of Commonwealth expenditure on particular drugs

Procurement as outlined in the DAS Guidelines is an integral part of good resource management, program support and delivery of any Government program.

The Guidelines recognise that well defined and consistently applied procedures and processes are very important for achieving good results and continuous improvement. The Government's objectives in procurement in all its purchasing programs, as noted in the DAS Guidelines, involve striking a balance between achieving continuous measurable benefits in value for money terms (on a whole life basis), benefits of industry development, and satisfaction in the supplier community with the operation of the Commonwealth's procurement system overall.

The procurement Guidelines see the program manager as having day-to-day responsibility for purchasing decisions. The procurement cycle in the Guidelines gives guidance on essential processes for successful procurement, in particular for ensuring value for money.

Table 5.1 lists the stages in the DAS procurement cycle[25] and the comparable element of the PBS process. PBS processes are closely equivalent to the DAS standards, except with the first steps in the purchasing process:

- in the first three stages of the cycle, which are essentially about setting priorities and planning, the DAS Procurement Guidelines envisage purchasing agencies will take the purchasing initiative. PBS procedures in contrast are essentially reactive, leaving the initiative to the seller, in this case the manufacturer or importer of the drugs;

- in selection and contracting (stages 4 to 9) DHFS procedures (in TGA and PBS) have equivalent processes to the DAS Guidelines. For example, although DHFS does not 'qualify competent suppliers' or 'prepare specifications' as such, it does rigorously vet the quality and safety of the goods (through the TGA process) and, through the cost effectiveness guidelines, provides a rigorous format amounting to specifications;

- in addition, elsewhere in the selection process, the preliminary evaluation (ie, at PES and ESC stages) of the goods on offer for purchase is strong and rigorous, employing a mix of well defined and

[25] *Interim Procurement Guidelines*, part 3, p115.

consistently applied procedures and advice from groups of experts in appropriate fields. Notwithstanding this, as outlined below, the ANAO considers that some improvements could be affected in the later stage of the evaluation and selection process to keep it abreast of more recent developments ;

- PBB and PBAC both have mechanisms for consultation with industry in the lead-up to the sponsor's application and as a follow-up. Though individual companies may raise questions about the effectiveness of these processes, the ANAO notes that they are recognised parts of the system and on the whole industry finds the consultation useful;

- in so far as the purchaser/provider 'contract' is managed in conventional terms (stage 11), this is the function of the Health Insurance Commission (HIC). The HIC has day-to-day management responsibility for drug procurement and payments. The PBS does have a continuing role through a rolling annual review of prices and a follow-up evaluation of health outcomes in relation to certain selected drugs (stages 10-12).

Against that background, the ANAO suggests that DHFS explores the following as a means of making the PBS listing process even more effective:

- in the early priority setting and planning stages (stages 1 to 3), ANAO notes that the demand driven nature of PBS places DHFS managers in a difficult position in regard to the conventional means of priority setting and planning. Notwithstanding this, ANAO suggests that DHFS explores ways to minimise this disadvantage.

- in the negotiation process ANAO notes that DHFS accepts the sponsor's price as a starting point which, in effect, sets the agenda. This occurs in the way in which the sponsor nominates a selling price which it says is cost effective. Subsequent discussions hinge around this nominated price. However, any drug may be 'cost effective' through a range of prices, either side of the sponsor's offering price - from a high price where it is only just cost-effective down to the price below which the sponsor is not willing to provide the drug. In the present context, the PBAC and PBPA will question prices if these are considered excessive. The basis of this judgment usually is the relationship to other drug prices. This can be effective as a negotiating strategy. However, the PBS could place itself in a stronger

bargaining position if it were to have an effective way of calculating the health benefits from the Government's point of view in order to calculate and develop its appropriate counter bid; that is, how much the benefit is worth to PBS in health terms (possibly in reference to estimated benchmarks for allocative efficiency) ;

- concerning the management of the contract between purchaser and provider, ANAO has noted above that this is largely a HIC responsibility. However, PBB does have responsibility for the review and evaluation functions. To fulfil that responsibility ANAO notes that the Drug Evaluation Sub-Committee (DUSC) has an important evaluation function in monitoring a sample of drug use which PBAC has directed it to follow-up, mainly from the perspective of clinical effectiveness. This monitoring can be made more comprehensive, and proposals for this are considered further below.

Reconsideration of the current approach to purchasing offers a new approach to reviewing the current high rate of growth in PBS outlays.

5.3 The PBS (PBAC/PBPA) decision-making

ANAO reviewed the roles of PBS advisory committees in the use of evidence from economic analysis and in the rigour of pricing decisions.

Use of evidence from economic analysis

Documentation provided to advisory committee members by the DHFS Secretariat was comprehensive and thorough . The PBAC and ESC timetables, although tight, have provided sufficient time for consideration of the material by members. However, it must be acknowledged that this depends on a high degree of dedication by members in working through the large amount of information involved. Similarly, the system of having two advisory committee discussants for each drug application worked well, with the quality of the presentations to the PBAC meeting overall of a very high standard. PBAC consideration of clinical and pharmaceutical issues appeared lengthy and thorough.

In Chapter 4 the ANAO noted DHFS' major effort to produce information on the cost effectiveness of drugs proposed by industry for PBS listing. The ANAO consultancy on cost-effectiveness confirmed that this process and the information which it produces is high quality, and

that it provided information of great usefulness in decision making. ANAO suggests, however, that there are several ways in which these benefits can be further enhanced.

Currently, the listing process generates cost effectiveness information through sponsors' provision of data and analysis as required by the PBS Guidelines. This is then evaluated by PES and by the ESC. Comment from these two bodies is provided, together with the sponsor's rejoinder to the PES comment, to the PBAC for its consideration. The PBAC recommendation based on this information, together with the PBB summary of relevant information, is then provided to PBPA for it to advise the Minister on pricing of the drug under consideration.

ANAO noted that industry raised some concern about the appropriateness of this process. Industry representatives observed that the IC Report recommended that ESC - as the main expert body in the PBS listing process - should make recommendations to PBPA as the prime pricing authority rather than to make recommendations to the PBAC . ANAO found some merit in this suggestion.

Under the current structure, such a suggestion would be consistent with the pattern laid down in relevant legislation and in Cabinet decisions. That would be because the first factor which the PBPA is required to take into consideration in its deliberations on pricing issues is the PBAC's advice. This is certainly appropriate in terms of PBAC's views on the clinical and pharmaceutical benefits of drugs. However, it can be argued that there are advantages in PBPA having direct information on economic and cost effectiveness issues from ESC rather than having it filtered through PBAC.

As part of the audit, ANAO compared, in a small sample of applications, the ESC written advice to PBAC with information and recommendation from PBAC to PBPA and the subsequent use by PBPA of this information .

ANAO noted that the amount of information provided by ESC to PBAC was considerable and detailed. However, the amount of this information which was transmitted to PBPA was considerably reduced. PBPA does not receive all available economic and pricing information but a summary of the economic and pricing data through the PBAC. In effect, PBPA forms its judgments on summaries of the original ESC information, both on the clinical-pharmaceutical and the economic-cost effectiveness issues. In addition, of the various factors which PBPA takes

into consideration, by far the most consistently and fully provided is that relating to PBAC's advice, while information on other factors was more variable.

While not conclusive, this information suggests that there may be a mismatch in the application of expertise in the evaluation and selection processes. Both clinical and economic factors need to be considered by expert bodies. However, economic matters are dealt with in the main by PBAC which is pre-eminently a clinical-pharmaceutical body with no health economist, not by the PBPA which is intended to be the expert pricing body. Another way to describe the processes is that the submissions and comment from PES and ESC develop the raw material for sound decision making, but this is under-utilised in that it is not considered in full by an expert body capable of understanding and making judgments on the information.

Recommendation 9

ANAO recommends that the PBPA should be provided with additional economic and pricing analyses and data to better inform its decision making.

DHFS Response:

Agreed with qualifications. DHFS considers that the type of additional information to be provided should be assessed as part of the review in response to recommendations 11 and 14. In the meantime, DHFS will arrange for the PES evaluation and the ESC advice, where available, to be provided to the PBPA. To minimise the risk of a duplication of the roles of the PBAC and the PBPA, the PBPA will need to consider this information in conjunction with and not in isolation to the PBAC advice.

The composition of the advisory committees

The membership of PBAC predates introduction of the evidence based approach and economic analysis. PBAC's composition reflects considerations when the National Health Act 1953 was being drafted in the 1950s. Different stakeholders raised with ANAO the issue of whether the current composition of and relationships between committees remains appropriate. ANAO notes that amendments to the membership have been undertaken recently by the addition of a consumer

representative. In addition, DHFS is considering further changes to PBAC's composition.

In order to maximise use of the Department's and its advisory committees economic analyses, ANAO suggests addition of health economists with pharmaceutical knowledge to the PBAC.

PBPA has representation from the pharmaceutical industry and from consumer organisations. Its membership also could be strengthened by addition of a health economist to participate in discussions on drug prices and costs.

Recommendation 10

ANAO recommends that DHFS reviews the roles and composition of PBS advisory committees to ensure that, in addition to the present high level of consideration of clinical and pharmaceutical issues, the best use is made of economic data in applications for PBS listing.

DHFS Response:

Agreed. A review of the appropriate composition and skills of the PBAC is under way. Consideration will be given to formally including a health economist on the PBPA.

In considering a possible restructure, DHFS could bear in mind the need to strengthen the role of advisory committees in advising on broader issues of the overall cost of the PBS as well as on the price of individual drugs.

ANAO is aware that there could be a variety of possible options for a restructured advisory committee arrangement. The following is only one option provided as an illustration of a relatively simple arrangement which attempts to retain what is working well at present and to introduce improvements where advantageous.

This option aims to give primacy to the purchasing function and provide a balance between the need for clinical/pharmaceutical and economic-cost effectiveness advice. ANAO suggests in the place of both PBAC and PBPA, a single advisory committee. This 'Pharmaceutical Benefits Purchasing Committee' could have two major technical subcommittees,

one clinical/pharmaceutical, the other economic. The proposed Pharmaceutical Benefits Purchasing Committee could combine both clinical and a greater amount of economic expertise in one peak body to advise the Minister on pharmaceuticals purchasing and value for money issues.

The process could then work as follows:

- applications would be lodged as at present with the PBB for preliminary scrutiny and evaluation by the PES;
- then passed to a Clinical Sub-Committee (CSC) to consider the application, together with PES comments and to make recommendations on comparative pharmaceutical advantages;
- then to an Economic Sub-Committee to consider the application, PES and CSC comments, plus industry comment on CSC's recommendations to make recommendation on economic matters;
- then to the Purchasing Committee consisting of clinicians, pharmaceutical experts and health economists;
- final negotiations by PBB and then recommendations to the Minister as at present.

Advantages of such a system could include:

- the major decisions on pricing and cost issues would be made by an advisory body with a more balanced representation of clinicians and health economists;
- clinical/pharmaceutical matters and economic matters would each be considered by expert sub-committees with clinical preceding economic. If clinical advantage were not demonstrated, the application could be returned to the sponsor without unnecessarily going to ESC;
- some time saving by focusing on one major committee with two major sub-committees rather than having two major committees; and
- the minor time savings realised compared to current arrangements could be used to shorten the duration of the process, or be used to allow for applications rejected by one of the sub-committees to be resubmitted by sponsors in time for the scheduled Purchasing Committee meeting.

Consistency in decision making

Several industry and professional group representatives raised the issue of the need for consistent application of selection criteria across all type of drugs. One view was that drugs for some indications, such as those used for treating HIV-AIDS, had more favoured paths through PBAC than others. It was believed that these more favoured paths opened because of the success of lobby groups. A submission from the Australian Federation of Aids Organisations (AFAO) argued that its political lobbying was essential to ensure that the Health Portfolio gave a high priority to life saving drugs[26]. AFAO further argued that its lobbying was an indication that sound administrative processes were not functioning in the Government's policy on the subsidisation of pharmaceutical prices.

The view that lobbying is essential is widely held but one that is difficult to substantiate. Yet there was a perception among professional associations and some industry representatives of a need to reduce the influence of lobby groups because, if they had excessive influence, treatment inequities could develop between some health consumers and others. ANAO did not seek data on the activities of lobby groups in the PBS recommendatory processes. However, in the case of those pharmaceutical products which have a clear life saving potential, there may be sufficient reasons for DHFS-PBAC to explore the possibility of establishing a 'high priority drugs' category with special processes. Such a step may reduce or at least channel lobbying to have particular products listed. The PBAC is already taking some steps down this path.

As discussed in the previous chapter, the ANAO advocates the introduction of allocative efficiency in evaluation of drugs, (see Appendix 1). Such a step would help in removing both the reality and the perception of lack of consistency in decision making held by some lobby groups.

5.4 Reviews - for price revision and for improved decision-making

The PBS/PBPA price revision process

The following comments arise out of ANAO's observations of two PBPA meetings and, in particular, of the pharmaceutical products pricing review documentation and its consideration at those meetings.

[26] Australian Federation of AIDS Organisations, Submission to the ANAO, January 1997.

ANAO noted that DHFS undertakes to have a review of each drug listed on the PBS once each year. This review is conducted on a progressive basis with a proportion of the drug groups being considered at each meeting so as to achieve full coverage within the specified time frame. Drug companies are notified of forthcoming reviews to ensure that they are not taken unawares and miss opportunities to request price changes. The revision of part of the schedule is a major part of the proceedings of each PBPA meeting.

PBPA's documentation for these reviews is clear and concise; the ANAO's observers had little problem understanding the system with minimum previous contact. The review documents listed the pricing history for each drug together with others in the same group which made for easy comparison with similar drugs.

Overwhelmingly the proposed revisions arose from manufacturers' requests. ANAO was not aware of cases where the revision exercise was in response to proposals from DHFS itself. ANAO noted that at the July 1997 meeting that there was a high proportion of drugs where manufacturers were not seeking price increases.

ANAO considers that the pattern wherein most requests for price variations come from manufacturers reflects the history of the process. The price revision process dates from days of high inflation when the Government was able to reap steady reductions in real prices unless industry requested increases to compensate for price erosion by inflation. The process depends essentially on suppliers making requests for price increases. Nowadays, in a period of low inflation this approach may be expected to produce fewer requests from industry since the prices remain valid for a longer period.

ANAO considers there may be value in DHFS rethinking the appropriateness of this process. If the Government is to realise economies as older drugs become comparatively less effective (and companies have succeeded in recouping their development costs), there may be scope to adopt a more active approach in the price revision process. DHFS should decide, on a more regular and systematic basis, which drugs may no longer be of the same value for money as they were in previous years, and either to negotiate a reduced price or remove drugs from the PBS Schedule altogether. This suggestion is made in the awareness of the need to avoid unintended consequences of removing a drug which may prompt GPs to prescribing a substitute drug still on the PBS list, which may be either more expensive, less appropriate, or both.

Evaluation of previous decision making

An essential part of any program, notably of a procurement program, is to follow-up and evaluate the effectiveness of previous decisions. ANAO noted that part of the function of the Drug Evaluation Sub-Committee (DUSC) is to evaluate the quality use of drugs. However, ANAO notes that DUSC, in acting on behalf of the PBAC, monitors the use of drugs from a clinical rather than a purchasing point of view.

Evaluation of previous decisions in the PBS context has a general relevance in terms of checking on the validity and reliability of the current decision making processes so as to identify opportunities for further improvements.

A more specific purpose relates directly to pricing levels agreed with the sponsors.

PBAC/PBPA decisions on fair and appropriate levels of pricing are based on cost effectiveness considerations which themselves are predicated in part on estimates of likely use. In cases where the use turns out to be greater than estimated in the cost-effectiveness analysis, companies could be reaping windfall profits and the Commonwealth could be paying more than necessary for these drugs.

PBPA/DHFS at present negotiates some price/volume trade-offs for certain higher priced drugs where there is some uncertainty about the likely use. ANAO suggests that the comparison of estimated use with actual use be made more comprehensive and systematic in order to indicate whether a more frequent and more effective use of price/volume trade-offs would lead to fairer and more reasonable pricing decisions.

Recommendation 11

ANAO recommends that DHFS develops its systematic monitoring of the use and the total costs of pharmaceuticals on the PBS, in order to establish whether the basis on which particular prices were agreed with manufacturers remains valid.

DHFS Response:

Agreed. The cost implications of new benefits are already considered by the PBAC and the PBPA. The Drug Utilisation Sub-Committee monitors use and cost against these predictions. The PBPA also undertakes

monitoring for price/volume arrangements and in areas where the price of drugs is set in relation to the average monthly treatment cost of alternatives. The monitoring in both areas is increasing.

5.5 PBS and promotion of pharmaceutical benefits to GPs and the public

The National Health Act contains restrictions on the Department's provision of information to health professionals and the public about, among other things, the relative merits of different drugs. One significant effect of these restrictions is to hamper the Government as purchaser of pharmaceutical benefits from maximising the benefits which could be gained by the public from the use of drugs.

DHFS cannot use its position as the principal purchaser of drugs in the Australian market to maximum effectiveness to improve national health because it cannot advertise the comparative benefits, limitations and optimum uses of the products it has bought. ANAO considers that DHFS could find benefit in investigating how the National Health Act might best be modified to enable it to promote the benefits of its purchases to the public.

Recommendation 12

ANAO recommends that DHFS explores ways in which the Commonwealth can better inform prescribers and users of the benefits, limitations and costs of the drugs available through the PBS.

DHFS Response:

Agreed. The secrecy provisions of the National Health Act preclude the Government from releasing any information considered during the listing process. The Department is discussing with the APMA how information which would be in the public interest can be provided.

5.6 Some cost containment issues

Specific responsibility for cost containment lies with the Minister and the Department itself rather than with the major advisory committees. PBAC is required to advise the Minister on cost considerations, but only

in relation to the cost effectiveness of particular drugs proposed for inclusion or already included as a benefit under the PBS, and on the effects of the addition of these particular drugs on the overall cost of PBS. However, PBAC does not advise the Minister on the implications of the aggregated impact of additions of several drugs over a period of time - say over the course of a particular financial year.

Similarly, the original terms of reference provided by Cabinet for the PBPA require it to maintain a continuous review of prices and conduct negotiations with companies about proposed prices (both of drugs proposed for PBS listing and changes to existing listings). However, the factors which the PBPA is required to take into account in its advice to the Minister similarly do not mention the overall cost of the PBS and can be read to relate primarily to the price and cost of individual drugs. Only in the Annual Report (for the year ending 30 June 1996) is the Authority's objective seen to incorporate securing 'a reliable supply of pharmaceutical products *at the most reasonable cost to the Australian taxpayers* and consumers'. Even this is not clear as to whether it refers to the price of individual drugs or to the effects of particular drugs on the cost of the whole PBS.

This means that, in providing advice on the price of particular drugs under the current system, advisory committees are not obliged at the same time to provide advice on overall cost implications of the aggregation of a series of additions to the PBS schedule to the relevant Minister.

Recommendation 13

ANAO recommends that DHFS considers ways to strengthen the roles of advisory committees in advising the Minister on the cost implications of total PBS listings, by making this requirement more specific.

DHFS Response:

Agreed. This information is already part of the economic requirements and the information is provided to the Minister. The reports of the PBAC and the PBPA state the cost implications for the listings of new drugs and for the changes to the listing restrictions. This matter will be considered in relation to recommendation 11.

Following its regular review meeting, the PBPA also advises on the cost implications for price changes (both increases and decreases). PBB also prepares regular reports on the PBS expenditure and how this relates to prior years.

6. ACCOUNTABILITY AND SOME RELATED ISSUES

6.1 Introduction

A critical criterion for the audit sought to establish whether the DHFS was accountable for the administration of the PBS. This chapter looks at both internal accountability, within the Department to the Minister, and external accountability to Parliament and stakeholders outside the Government. In addition, the chapter addresses issues of DHFS' resourcing of the listing process, and considers whether an appeals mechanism against listing decisions would be appropriate.

6.2 Accountability

Internal accountability

The Portfolio Budget Statements establish the basis for accountability to Parliament and set out:

- the objectives and strategies developed for portfolio programs;
- information on budget measures affecting portfolio programs; and
- the performance indicators and evaluations to be used to assess the performance of portfolio programs.

The ANAO examined the Portfolio Budget Statements and Departmental annual reports, expecting that the objectives set out in the PBS for 1995-96 would be reported against in the annual report for 1995-96 (the latest available at the time of the audit). Some performance information was available but was insufficient to allow a complete assessment of the program's outcomes.

In the 1997-98 Portfolio Budget Statement, ANAO found indications that the DHFS has laid the foundation for a more thorough disclosure of its performance reporting against its objective and goals in future. The PBS now provides the Parliament and other interested readers with a list of indicators, targets and the information source and reporting frequency for each, to facilitate a performance assessment of the program. This has been done for the effectiveness, efficiency, quality and equity issues relating to the program.

The ANAO considers that this promises to provide timely and comprehensive information in an innovative way to satisfy accountability requirements to the Parliament. These details will be useful to PBS stakeholders.

External accountability

As well as the formal requirement to report program objectives and results to the Parliament, ANAO considered the nature of any responsibility for DHFS to be accountable to other stakeholders. These include the pharmaceutical industry, drug consumers and the Government.

In passing, it is important to note that as well as periodic accountability which is addressed below there is also accountability of a more continuing nature. This takes the form of the need to provide information to industry on PBS decisions, and the issue of transparency to both industry and consumers. Further discussion on these issues can be found in Chapter 4.

Accountability between purchaser and provider

Much of the earlier commentary on industry's preference for more information about listing processes is relevant to the Department's accountability to industry. The major determinant of any such accountability is that the Government, through the Department including the PBAC, is a purchaser of products from the private sector. The pharmaceutical industry is the provider of those products. This commercial relationship means that the nature of accountability between the Department and industry may differ from the accountability of the Department to Parliament and to Government. In a commercial relationship, if the purchaser is accountable at all to the provider, then the purchaser is accountable for clear communication of the purchase request, where the latter includes the agreed price, and payment of the correct amount on time.

Industry gave no indication that the purchaser, which was the Department on behalf of the Government, provided insufficient information about what it wanted to buy from industry. Nor did industry complain about any late or incorrect payments by the Department. If the provider is accountable in a commercial relationship, then the provider must provide the agreed products on time and

according to the agreed price. The Department, as the purchaser, raised no complaints with ANAO about these matters.

In addition to these considerations, there are those arising from the DAS Interim Procurement Guidelines, in which one of the Government's objectives is noted to be 'satisfaction in the supplier community with the operation of the Commonwealth's procurement system overall'. The core principles include open and effective competition, and ethical and fair dealing, both requiring transparency, (see Chapter 4).

Accountability to consumer associations

As representatives of members of the public, the accountability expectations of consumer associations are to a large extent catered for by mechanisms for DHFS to demonstrate its accountability to Parliament. The collation of performance information as mentioned earlier in the chapter could serve to satisfy their expectations. However, some additional data on the listing process may need to be produced by the DHFS.

Because there was no information provided on the time taken to list a drug onto the Schedule, some consumer groups had a perception that all delays in relation to approval to list were the responsibility of the Department. As indicated in Chapter 3, this is not always the case.

Also, as discussed in Chapter 3, the ANAO found that DHFS has reduced the time taken to list a drug onto the Schedule, although there is scope for further reduction. Reporting these data to external stakeholders would allow them to assess the efficiency of DHFS' administrative processes. The perceptions of some professional bodies and consumer organisations that delays in listing drugs are solely the responsibility of the Department may be altered if these data were made available.

The ANAO found that there are also data in a DHFS quarterly report `Expenditure and Prescriptions' that could be useful to external stakeholders such as representative bodies of consumers and industry. The publication draws on data provided by the HIC and is currently mailed out to around 50 groups and individuals. Interested parties can be included on the mailing list by contacting the Department of Health and Family Services. As well as this publication the PBS runs a toll free information line. It provides ongoing assistance and information to the

public, consumer/community groups, health professionals, Parliamentarians, Government authorities and the media regarding the benefit items, safety net procedures and other matters relating to the PBS and Medicare.

A considerable amount of published information is already available to interested stakeholders. Assertions of insufficient accountability and transparency on the part of the Department of Health and Family Services about PBS, are, in part matters of misperceptions by stakeholders. There is be considerable benefit to be gained from DHFS' ensuring that industry and the interested public are made more fully aware of the performance information readily available to them, and for that performance information to be more widely promoted and disseminated.

To allow industry and consumers to be informed about the performance of the PBS, the ANAO suggests that the DHFS provides public information against the following indicators:

- the date the drug was approved for use and marketing by the TGA;
- the date the drug company's application for the listing of a product on the Schedule was received by the DHFS;
- the time taken to list drugs onto the Schedule, including
    - the proportion listed after consideration at a single cycle;
    - the average time taken for listing excluding delays for which the sponsor is responsible; and
- date approved by the Minister.

Recommendation 14

The ANAO recommends that the DHFS better inform industry and the public about the PBS listing process in order to reduce misconceptions about the role of the Department in this process, and facilitate understanding of the reasons behind the Department's purchase of pharmaceutical products.

DHFS Response:

Agreed. The Department will continue to inform industry about PBS matters including the listing process and consider how this process can be made more effective.

6.3 Performance measurement

As previously indicated, the ANAO used DHFS information to develop a database of submissions received from industry over the last five years (1991-1996). The database provides for the first time comprehensive, readily accessible information on each submission including:

- key dates in the approval process;
- identifying data on each application;
- pricing information;
- type of economic analysis performed; and
- the decisions made regarding listing and price.

While mainly intended to assist with this audit, the database could serve as a useful management tool. It was provided to the Department at the conclusion of this audit.

6.4 Outsourcing

As discussed earlier, in 1993 cost effectiveness analysis became an obligatory part of the requirements of sponsors seeking their products listing on the Schedule. Subsequently, the Department's work in reviewing sponsors' cost effectiveness data on new drugs grew considerably. At first the approach was to evaluate the cost effectiveness information on a selective basis. Soon after, the Department decided to evaluate all cost effectiveness data provided by pharmaceutical companies. Since the Department did not have sufficient staff for these evaluations, some of the latter were conducted by another agency on contract to the Department.

The ANAO noted that the selection procedure for this contract involved a selective tender open to ESC members as representatives of research units which might have an interest and capability for the work. Subsequently, in 1995, DHFS contracted the University of Newcastle,

Faculty of Medicine and Health Science, to conduct these evaluations which it has done through the Newcastle Evaluation Group.

The ANAO notes that this contract will expire in the near future. ANAO considers that DHFS could benefit from exploring the possibility of introducing greater competition into the contracting out of the evaluation of cost effectiveness data provided in applications for PBS listing. Competition could broaden the spread of evaluation expertise and experience available and further enhance evaluation methodologies.

Recommendation 15

The ANAO recommends that, in order to take advantage of the growing number of institutions capable of fulfilling the role, the DHFS broadens the competition for provision of evaluation advice to the Department on cost effectiveness data provided by pharmaceutical companies on their products.

DHFS Response:

Agreed. The Department notes the ANAO view that the Department could concentrate its resources more on policy work in the area of economic analysis and that the use of external evaluations services should be expanded, subject to available resources. While this is agreed to in principle, it would involve extra expense and depend on the availability of institutions with the necessary combination of skills and without potential conflict of interest that can fulfil the evaluation role adequately.

6.5 Appeals process (against PBAC decisions)

The Industry Commission acknowledged in its report on the pharmaceutical industry that PBPA price negotiations, by their very nature, were not amenable to formal review. The Commission argued that the lack of administrative appeal processes for PBAC recommendations reduced transparency and accountability.[27]

The main industry concerns expressed to the ANAO on the appeals issue centred around the transparency of the listing process rather than on any need for an appeals process. Industry representatives had different views on the merits of an appeals process. Some told the ANAO that recourse to an appeal would only serve to delay the listing processes.

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[27] Industry Commission, 1996, p.237.

There are only two grounds on which industry might seek to appeal:

- first, because of PBAC advice to the Minister not to list a drug on the Schedule; or
- second, because of dissatisfaction with the price at which the Department offers to purchase a drug proposed for listing.

Legal advice received by DHFS indicated that it is not appropriate for a decision on price or purchasing to be the subject of an administrative review by the Administrative Appeals Tribunal (AAT) *"because the scheme for declaring which drugs are to be PBS items (which includes taking into account PBAC recommendations) is already subject to Parliamentary scrutiny and disallowance. . (and) . . because declarations of the Minister under section 85 of the National Health Act are required to be tabled in the Parliament and be subject to disallowance (sub-section 85(2B)).* The AAT only has jurisdiction over administrative decisions.

However, the deliberations of the PBAC are subject to judicial review. Advice from the Attorney-General's Office states that in its deliberations the PBAC is required to adhere to the rules of natural justice. In that sense there is scope for an appeal against a recommendation of the PBAC under the provisions of the *Administrative Decisions (Judicial Review) Act 1977*. Sponsors would be likely to challenge a PBAC recommendation on these grounds in only a small number of cases because the time for an appeal may only serve to delay the listing process further.

On a more pragmatic level, the issue of an appeals avenue against decisions made by the PBAC was discussed at length at the 1993 annual meeting of the APMA/PBAC working party. The minutes of the meeting reflected the outcome of the discussion:

"It was generally agreed that a formal appeals process would delay companies in making a further submission to the PBAC and that the provision of clearer statements of reasons to industry and the growing transparency of PBAC procedures should alleviate the need for such a mechanism".

Canberra
P.J.Barrett

Date
Auditor-General

PART THREE

Appendices

APPENDIX 1

The Use of Cost effectiveness Analysis in the Pharmaceutical Benefits Scheme Listing Process

Digest of a Consultancy Report to the Australian National Audit Office by

Geoff Dixon and Geoff Vaughan[28]

The Pharmaceutical Benefits Scheme (PBS) provides the Australian community with state of the art health outcomes, but at large and growing cost to the budget. It is a $3 billion program and outlays are growing rapidly.

This rapid growth reflects a combination of

- an ambitious program objective - to provide timely, reliable and affordable access to necessary and cost-effective medicines, and
- a program environment in which the pace of improvement in drugs is rapid and the cost of new medicines is rising quickly.[29]

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[28] 'Consultancy on the Use of Cost Effectiveness Analysis in the Pharmaceutical Benefits Scheme Listing Process: Report by Geoff Dixon and Geoff Vaughan', 28 July 1997.

[29] The budget cost of the PBS is particularly sensitive to the pace of improvement in drugs because

- in common with programs in the education and social security areas, use of PBS drugs is demand driven. Within the framework of the Scheme, GPs are free to prescribe new drugs as necessary and of the type necessary (including newer and more expensive variants)
- somewhat unusually for Commonwealth programs it is also (in a qualified sense) 'supply driven'. The PBAC will normally recommend listing any new drugs proposed by a company which have higher levels of outcomes (and are more expensive per treatment) as long as the cost *per unit of extra outcome* represents value for money.

In the current economic environment there is clearly a growing policy tension between budgetary control and improved health outcomes in Australia. Management of this tension is a key challenge for the program, and a criterion by which the existing drug listing arrangements must be judged.

In this regard the PBS drug listing program is a flag bearer among Commonwealth programs in terms of the use of sophisticated decision techniques. Australia is the first country in the world requiring data on cost effectiveness prior to the reimbursement of new pharmaceuticals and considerable interest attaches to the benefits flowing from this requirement. An earlier criticism that the Commonwealth government demands much more in the way of cost effectiveness analysis than other countries in proposals for drug listing has become less cogent as other countries have increasingly followed in Australia's footsteps.

Moreover the Pharmaceutical Benefits Advisory Committee (PBAC) and the Department have shown a clear interest in further development of the decision process for listing new drugs, and steps are in hand for exploring options for further improvement. This promises direct benefits because of the large cost of the program, and the rate at which that cost is growing. It may also bring indirect benefits through the pioneering of improved decision-making techniques which might be relevant to other Commonwealth programs in a diverse range of portfolios.

2. Value for money in the drug listing process

Whether value for the $3 billion spent on the PBS is maximised depends in the longer term on the efficiency of the listing process for new drugs.[30] Given the scale of the program and the rate at which outlays are growing even apparently minor defects in listing procedures could result in substantial shortfall in value for money building up over time.

The move from an opinion based listing process to an evidence based process in 1993 has been central to the achievement of value for money in the listing process. In 1993 it became mandatory for drug listing proposals to supplement information about the clinical effectiveness of new drugs with information about the cost effectiveness of the proposed drug relative to existing PBS treatments in the same therapeutic field.[31]

The PBAC will normally recommend listing a proposed drug where

- it will produce better results than the listed drug it is likely to displace, **and**
- the extra cost to the PBS over the existing drug is acceptable.[32]

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[30] How can the efficiency of the listing process be defined? According to the priority outcomes for the scheme contained in the 1997-98 Portfolio Budget Statements it must ensure that new drugs listed on the PBS are

- clinically acceptable
- necessary for the treatment of relevant conditions
- cost effective, in the sense of being lower cost per unit of outcome than existing drugs listed for the treatment of the condition.

The first is necessary to provide reliable access to medicines and the second and third to ensure maximum benefit from spending on the Scheme.

[31] The previous opinion based process used expert opinion whereas the evidence based approach uses (ideally) head to head clinical trials of the new drug with the existing therapy on the PBS Schedule.

[32] This discussion relates to submissions for the listing of drugs which offer improvements on existing PBS treatments. Where there is no improvement (the new drug is proposed on grounds of lower cost or as a generic) the value for money issue is more straight forward.

These two criteria **together** determine whether a proposed drug increases value for money for PBS outlays. This dual criterion is summarised in a cost effectiveness ratio which measures the extra cost of treating patients with the proposed drug (rather than the listed alternative) per unit of extra benefit enjoyed over the listed alternative.

The use made of this dual criterion by the PBAC was examined in a retrospective analysis of PBAC decisions undertaken by Bethan George for the Department of Health and Family Services.[33] She found that

- in cases where a proposed drug was superior to the treatment already listed under the Scheme the PBAC has been reluctant to recommend listing the new drug if the extra cost to the PBS (over the existing treatment) exceeds $78 000 per unit of additional benefit (quality adjusted life year)
- drugs for which the extra cost was below $37 000 were normally accepted.

In cases where the extra cost to the PBS (over the existing treatment) fell between $37 000 and $78 000 there was a less clear cut relation between the position of particular drugs on the league table of cost effectiveness. George attributed this to PBAC's consideration of factors other than efficiency in producing health gain at minimum cost, uncertainty on the part of the Committee about the accuracy of the estimated incremental cost effectiveness ratios or possibly to inconsistent decision-making.

Although the PBAC is not formally tasked with observing a budget constraint when it is recommending the listing of new drugs, reference to a threshold level of the cost effectiveness ratio in deciding whether to recommend listing is

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While displaced drugs are not removed from the Schedule, there is a presumption that GPs will - prescribe the most cost effective drug on the Schedule, and steps are in train to encourage this.

[33] George, B. 1996, Cost-effectiveness League Tables: Their Use in the Decision to List a Drug on the Australian Pharmaceutical Benefits Schedule. This document is currently unpublished. However a published version will be available shortly from DHFS.

effectively introducing a budget constraint into its decision making process. New drugs for which the extra cost (in relation to the extra benefit) is above the threshold are implicitly treated as not adding value for money to PBS outlays.

3. Does the listing process ensure good value for the $3 billion spent on the program?

To assess whether the introduction of cost effectiveness information about new drugs to the listing process has led to an improvement in value for money from the program it is useful to separate PBS listing procedures into three stages:

- the adequacy of the PBAC Guidelines for company submissions proposing new drugs for PBS listing. These outline the extent and nature of cost effectiveness information requested from the company in its submission;
- the extent to which company submissions adhere to these Guidelines in preparing this cost effectiveness information; and
- the extent to which the PBAC exploits the full potential of this cost effectiveness information in arriving at its listing recommendations.

A weakness in any one of these three stages can undermine the effectiveness of the other two stages. The following assessment of the use of cost effectiveness information in the listing process looks at the performance of the listing process at each of these three stages in turn.

However an overall judgment is that the quality of advice available to the PBAC on the cost effectiveness of drugs proposed for listing is excellent. This reflects the high quality (including by international standards) of cost effectiveness reporting required by the Guidelines, together with the rising plane of conformance by companies with the Guidelines (the first two dot points above).

4. A. The first link in the quality chain: adequacy of the Guidelines

The Guidelines provide a 'template' for companies preparing cost effectiveness information for their submissions. They are, in effect, a highly succinct handbook on the combining of clinical and economic analysis which must command respect. It would be impossible to argue that the PBAC has failed to clearly identify the standards it is seeking in company submissions or to provide a high level of assistance to companies in meeting those standards.

The requirement that a new drug be rigorously compared with the existing PBS treatment and its advantages be shown to be acceptably cost effective ensures that over time there is a 'leap-frogging' effect in value for money

- existing drugs in each therapeutic field are displaced by a more effective successor only if any increase in cost is acceptable
- in time the successor will itself be displaced by a yet more effective substitute (again provided the increment in cost is acceptable).

The cost effectiveness requirement effectively precludes clinically effective but cost ineffective drugs being listed. It is important to note that these improving outcomes over time come at an ever increasing cost to the PBS.

There are however several areas in which the Guidelines are capable of improvement. These are discussed in the next five sub-sections.

4.1 1. Comparisons of cost effectiveness across therapeutic fields

The major shortcoming in the 1995 Guidelines perceived by the consultants is that they allow companies considerable diversity in the choice of outcome measure used to compare the new drug to the existing PBS treatment. Thus the outcome measure chosen by the company

- may be specific to the particular therapeutic field in which the new drug will be used (such as, for coronary thrombosis, the number of subjects with specified level of left ventricular function following use of the new and existing drugs) **or**
- may apply across a broader range of therapeutic fields, such as quality adjusted life years gained as a result of listing the new drug. This is an outcome measure which could apply to a diverse range of drugs across different therapeutic fields.

Why is this degree of 'agnosticism' on the part of the Guidelines in regard to choice of outcome measure seen as a shortcoming?

The main reason is that PBAC listing recommendations will only maximise value for money from the program if they are systematic through time and across therapeutic fields. A lack of consistency between listing recommendations at different points in time or for different therapeutic fields means that value for money from the program is less than it would otherwise be under best decisionmaking practices.[34]

In this regard outcome indicators which are specific to the therapeutic field of the new drug are less helpful to the PBACs efforts to balance value for money across therapeutic fields than the use of more general outcome indicators.

For example, suppose the PBAC has before it proposals from two companies to list a new HIV drug and a new drug for reducing hypertension. The outcome measure in the HIV submission is the proportion of subjects in the trial with a maximum weight gain greater or equal to a threshold level over a specified period, while the outcome measure in the anti-hypertensive submission is the reduction in the number of subjects experiencing a stroke.

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[34] Inconsistent decision making would mean that some rejected drugs may offer a greater improvement in health outcomes in relation to their cost to the PBS than other drugs recommended for listing. The Department and PBAC are aware of this point and the study by Bethan George is a first attempt to review the level of consistency across past PBAC recommendations.

While it is possible for the PBAC to conclude whether each drug is more cost effective than the existing PBS treatment in the **same** therapeutic field (since a common outcome measure is used to compare the proposed drug with the existing treatment in the field in each case) it is not possible for the PBAC to determine whether the proposal to list the HIV drug is better value for money than the proposal to list the anti-hypertensive.

For example, it cannot be determined whether a cost effectiveness ratio of (say) $55 000 per improvement in outcome for the new HIV drug is better or worse value for money than a cost effectiveness ratio of $45 000 per improvement in outcome for the new anti-hypertension treatment. While it is possible for the Committee to rank listing applications **within** a particular therapeutic field where outcome indicators are field specific, it is not possible for it to make decisions about which is best value for money across therapeutic fields.[35]

Where different listing applications use different outcome measures it is not possible to be sure that a drug recommended for listing in one field would always add more value for money than a rejected drug in another field.

In order for cost effectiveness comparisons to be made across therapeutic fields it is necessary for all listing applications to use common outcome measures. A common measure which is widely accepted and used is the quality adjusted life year. The Guidelines recognise that this is an outcome measure which companies **may** use for the cost effectiveness analysis (and increasingly do so), but essentially leave it to the company to choose

[35] In the terminology of the consultant's report, ranking of different drugs **within** a therapeutic field is necessary to ensure technical efficiency of the listing process and ranking across therapeutic fields ensures allocative efficiency of the process. Outcome indicators which are specific to the therapeutic field allow PBAC listing decisions to be technically efficient but not allocatively efficient. Had both applications used the same outcome measure (such as quality adjusted life years) the two drugs **could** be ranked in terms of cost effectiveness, even though they are to be listed for use in different therapeutic fields.

whether the outcome measure adopted is specific to the therapeutic field or more general in nature. The Guidelines should go further by recommending that wherever possible outcome measures which are general across therapeutic fields should be used.[36] This leads to

Conclusion 1: the use of more uniform outcome measures should be explored in the course of developing the next revision of the Guidelines.

4.2 2. Use of cost benefit analysis

There is a second area in which consideration might be given to improving the Guidelines. In the 1995 version the use of cost benefit analysis in company submissions is discouraged, and cost effectiveness analysis encouraged.[37] This reflects a concern on the part of the PBAC about the tendency in many cost benefit studies to 'monetise' all the costs and benefits involved and a doubt that this is appropriate in the health area.

However the use of a non monetary measure of benefit such as quality adjusted life years is quite acceptable in cost benefit analysis and the key difference between cost effectiveness analysis and cost benefit analysis is arguably slightly different. If outcomes were to be measured in terms of quality adjusted life years gained, the difference between the measures under the two methods are as follows:

- cost effectiveness analysis measures benefits in a ratio form, viz. the cost to the PBS per quality adjusted life year gained by listing the drug; and

[36] It must be recognised that generic outcome measures such as quality adjusted life years are an area in which there is considerable research activity and some way to go before there is complete agreement on a common approach. It may therefore be some time before it is feasible to require the use of quality adjusted life years in **all** therapeutic fields.

[37] The scaling factor is normally an estimate of the numbers of treatments using the new drug should it be listed (which is already requested in the company's submission as part of the analysis of the financial implications for the PBS of listing the drug).

- cost benefit analysis measures benefits in an aggregate form, viz. the total quality adjusted life years saved by listing the drug and the total additional cost to the PBS.

Use of a cost effectiveness ratio therefore has the disadvantage that PBAC is obliged to mentally scale up the ratio to compare the total benefit from listing the drug with the total cost to the PBS. Moreover when the Minister is making decisions about listing against the background of the need to manage total spending on the Scheme an estimate of total benefits and total estimated cost is required in order to obtain the greatest benefit from the PBS budget.[38] This leads to

Conclusion 2: the potential contribution of cost benefit analysis (which is currently discouraged by the Guidelines) receive early attention, particularly in regard to

- **use of measures of absolute net benefit rather than cost effectiveness ratios, and**
- **the use of willingness to pay techniques for measuring benefits.[39]**

4.3 3. Hard versus soft evidence

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[38] Cost benefit is superior to cost effectiveness only if there is a budget constraint. If there is such a constraint (as is invariably the case) it becomes important to take account of the **scale** of net benefits from each new drug in relation to its cost.

Suppose the budget constraint means that the Minister must choose between two options: **either** to list one drug with moderate cost effectiveness ratio and very large utilisation **or** a 'basket' of drugs some of which have higher cost effectiveness ratios but lower utilisation and others of which have lower cost effectiveness ratios and lower utilisation. The decision providing best value for money can only be made on the basis of the aggregate benefits of each of the two options, not on the cost effectiveness ratios. Cost benefit takes this scaling effect into account when it is used to prioritise the two options.

[39] 'Willingness to pay' is an approach to estimating the benefits from (in this case) new drugs based on the value the patient (or potential patient) places on the improvement in health brought about by the drug. The approach is receiving increasing attention in areas such as the analysis of environmental impacts of development proposals where it is desired to take account of individual preferences relating to a proposal in a structured manner.

In the present context it involves the use of carefully designed questionnaires to explore the maximum dollar amount the individual would offer for the improvement in health caused by use of the new drug. While it has the advantage of basing estimates of the benefits of a new drug on individual preferences about different health states, there are significant problems relating to questionnaire design and the distortive effects of differences in respondents' income levels on their money valuations of improved health outcomes.

A third area in which improvement in the Guidelines might be considered relates to a complaint by drug companies that the Department has an undue preference for hard evidence and attaches too little weight to expert opinion or other evidence of a 'softer' nature. As suggested by the industry, this could bias the PBAC decision process where particular benefits or costs are potentially important but are difficult to quantify, and hence are given insufficient weight in the decision process. This leads to

Conclusion 3: The importance of a decision process which enables the reconciliation of 'soft' evidence with 'hard' in a systematic manner needs to be

- **acknowledged by the Department**
- **explored in the strategy development process leading up to the next edition of the Guidelines.**

4.4 4. Sensitivity analysis

A fourth area in which improvement to the Guidelines might be considered relates to the use of sensitivity analysis. This is a powerful technique used in cost effectiveness and cost benefit analysis to explore the circumstances under which a proposed drug would **not** represent value for money. This would enable PBAC to assess the plausibility of such circumstances in arriving at a listing recommendation.

If companies were to make greater use of sensitivity analysis in their drug listing proposals PBAC would be less heavily reliant on the particular set of assumptions chosen by the company to justify listing the drug.(the cost effectiveness computations would be less of a 'black box'). This leads to

Conclusion 4: the Guidelines encourage more sophisticated sensitivity analysis by companies,

designed to increase the transparency of possible defects in cost effectiveness analyses.

4.5 5. Quantifying indirect effects

A fifth area for possible improvement in the Guidelines relates to the treatment of 'third party benefits' from the listing of more effective drugs. These relate particularly to freeing up of hospital beds otherwise occupied under existing treatment regimes and economic benefits due to earlier return to work by patients using the new drug.

The consultants share the Department's caution about incorporating these third party benefits in the calculation of the cost effectiveness ratio unless the precise adjustments taking place in the hospital system or labour market are carefully documented and the contribution of a decision to list the new drug to these effects clearly isolated.

One point of difference, however, relates to the request in the Guidelines that evidence of third party benefits be expressed in 'natural units, eg. extra days on the job because of accelerated recovery made possible by the new drug. This contributes to a multiplicity of 'metrics' in each company submission and complicates PBAC decisionmaking, which must also take account of ratios (the cost effectiveness ratio of the proposed drug) and intangibles such as equity issues conceptualised in absolute terms.

Conclusion 5: rationalisation of the metrics for the various direct and indirect benefit components associated with a new drug be addressed as part of the review of PBAC decision making processes leading up to the next revision of the Guidelines.

5.

6. B. The second link in the quality chain: the extent to which company submissions adhere to the Guidelines

While the Guidelines are a clear example of best practice among Commonwealth programs their contribution to value for money from the PBS depends on how far companies are able or willing to conform to them.

In this regard some sections of the industry argue that the Guidelines are both too onerous and too prescriptive in their requirement for cost effective analysis, and too difficult to follow.

For example, one company submission to the Industry Commission inquiry into the pharmaceutical industry criticised the exponential growth in information requirements in support of listing applications. These information requirements are seen as more demanding than those required for drug approval in other much larger overseas markets for pharmaceuticals.

However the industry is by no means monolithic in its criticism of the Guidelines. It was suggested to the consultants by one leading company that the information sought by the PBAC is soundly based in economic analysis. The substance of the comments made by the company are worth documenting

- the company's clients around the world, it suggested , are increasingly demanding economic justification for the drugs which it proposes to sell to them, as well as clinical evidence that the drug is effective
- the company is now undertaking economic appraisal of new drugs in parallel with their development cycle rather than leaving it to the point at which the drug is marketed

- it was suggested by the company that where a drug appears to offer little on the cost effectiveness front its further development might be terminated by the company (in contrast to the earlier practice in which clinical effectiveness was a sufficient justification for proceeding to market the drug).

This argument suggests that while the need for the company to prepare cost effectiveness analysis may in the past have been a burden imposed by the Australian government, this is now much less true. In a world in which there is a common trend on the part of drug purchasing agencies faced with spiraling costs to take account of the budgetary as well as the clinical implications of their drug funding choices, the identification of **both** the clinical and economic advantages of the new drug is increasingly part of the company's overall drug marketing strategy.

Economic information is increasingly collected by companies in parallel with the process of clinical testing and development.[40] Cost effectiveness analysis of a new drug being developed by a company would therefore be undertaken by the company regardless of whether or not it is required by the Australian government (albeit perhaps in a different format).

The diversity in company opinion about the justification for cost effectiveness analysis in new drug submissions may be related to the steep learning curve involved in combining economic with clinical analysis. In this regard there is general agreement that the quality of company submissions

[40] Some companies also argued that the presentation in the Guidelines is highly complex and very difficult to follow. Certainly there is a very high level of cross referencing within the document, although this reflected an attempt to provide companies with an encyclopedic coverage of the issues at a time when this was in effect a pioneering document and sole information source.

It seems likely that as companies move up their learning curve in regard to cost effectiveness analysis, the digestibility of the Guidelines will become less of an issue. The expansion of the health economics literature relating to cost effectiveness will also increase their access to supporting information. This said, however, there is a strong case when revising the Guidelines for drawing on the skills of those sections of the industry which have become more familiar with the sound use of cost effectiveness analysis in their submissions.

has improved markedly since the requirement to report on cost effectiveness was introduced in 1993. However some important issues remain, and are discussed in the next three sub-sections.

6.1 1. Monitoring the quality of company submissions

In order to assess the quality of cost effectiveness information provided by companies in response to the Guidelines a sample of thirty major submissions to the PBAC during 1995 and 1996 was considered by the consultants. The submissions involved twenty three different drugs, and included both new submissions to the PBAC and re-submissions.[41]

The results of the analysis are contained in Chapter 4 of the consultant's report. However the main point to note is that the cost effectiveness information contained in all but one of the company applications exhibited significant shortcomings of one or more types. Only three of the thirty applications were defect free, nine applications had one defect, eight had two defects, seven had three defects and three had four.[42]

This suggests that there are considerable shortcomings in the quality of cost effectiveness information currently being provided to PBAC by companies proposing drugs for listing. There is apparently considerable scope for further

[41] The methodologies used by the companies in preparing their cost effectiveness information included cost minimisation analysis (five submissions), cost effectiveness analysis (eighteen submissions), cost utility analysis (six submissions) and cost benefit analysis (one submission). Twenty of the drugs proposed in the submissions were accepted by the PBAC for listing on the PBS and ten rejected. The major analysis of the quality of the cost effectiveness information is provided by the Pharmaceutical Evaluation Section of the PBB in its commentary on each submission. The categories of defects are listed in Chapter 4 of the consultant's report.

[42] The most frequent category of defect in the cost effectiveness information presented by companies was associated with the **outcomes** claimed for the proposed drug. This reflected a tendency to (usually) claim more benefits for the proposed drug than was justified by the clinical tests, due to defects in modeling the implications of intermediate effects through to patient relevant outcomes, or the inclusion in the submission of claims which were insufficiently substantiated by the evidence contained in the submission.

improvement in the quality of cost effectiveness information provided by companies.[43]

The second point to note is that there is considerable variation between individual company submissions in the number of defects per submission. This ranges from five defects in the application with the worst score to zero defects in that with the best score, with a concentration between one and three defects.

This **dispersion** in the number of defects per submission is an encouraging sign. It is consistent with the view that best practice (as exemplified by the low defect applications) is an attainable goal rather than cost effectiveness analysis being intrinsically difficult for companies to do properly. Best practice (as exemplified by low defect applications) should therefore not be regarded as too big an 'ask' of companies.

The dispersion also suggests that some drug companies may have developed their skills in cost effectiveness analysis to a greater degree than others. This would be consistent with the view that the introduction to the drug listing process of the requirement to provide economic analysis alongside clinical analysis has involved a steep learning curve for companies, rather than reflecting an underlying malaise in the standard of applications per se, and that the quality of applications has improved greatly since 1993. This leads to

Conclusion 6: it would be desirable for the Department to establish an in-house time series database tracking the frequency and nature of defects in company submissions. This would be

[43] The presence of multiple defects in large numbers of submissions did not preclude a PBAC recommendation to list many of these drugs, and the Guidelines are clearly being administered flexibly rather than being used as a barrier to the listing of new drugs. However the existence of the defects in most of the submissions that were approved means there is less confidence that the PBAC recommendations are internally consistent and ensure best value for money for the program as a whole.

appropriately updated on an ongoing basis, as applications are assessed.

The existence of such a data base would enable the Department to

- assist and encourage companies experiencing difficulties with cost effectiveness analysis of their new drugs to improve the standard of their submissions; and
- refine the Guidelines to address recurring problems in applications from all companies. [44]

6.2 2. Updating the Guidelines

The Guidelines are undergoing an ongoing process of revision. Thus the 1995 Guidelines reflected experience with the operation of the 1993 version. A process is in train to develop the PBAC position on the next release of the Guidelines. This involves the preparation, under the guidance of PBACs Economic Sub-committee (ESC), of position papers and literature reviews as a basis for forthcoming discussion of the issues in the PBAC.

The time frame for the review of issues relating to the next edition of the Guidelines is fairly long drawn out, and on present Departmental thinking the new Guidelines are unlikely to be available until 1999. The revision of the Guidelines is therefore clearly a long term development rather than providing short term assistance to companies which are currently on a steep learning curve in regard to the use of generic measures of outcome in their applications.

In order to provide such short term assistance, consideration could be given to accelerating this review process. For example, this could involve publication of the Guidelines in loose leaf format,

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44 This data base would also serve as a basis for

- deciding whether remedial action is required to accelerate the improvement in the quality of cost effectiveness information provided by companies; and
- benchmarking cost effectiveness information provided by individual companies against the best practice submissions by other companies. As indicated above, there is evidence that some companies may be better able to handle the challenges posed by the preparation of cost effectiveness information, and this would provide a potential lever for raising the standards of other companies.

with individual chapters or sections being updated as issues are clarified within ESC and PBAC.

This would enable key issues to be addressed more quickly than in the time frame currently envisaged by the Department for full review and republication of the Guidelines. It would enable prompt attention to such key issues as whether there should be additional PBAC encouragement of the use of outcome measures which are common to different therapeutic fields, together with guidance on the strengths and weaknesses of willingness to pay measures in the estimation of drug outcomes.

It could also afford the PBAC the early opportunity (if it sees fit) to update its formal discouragement of cost benefit analysis in the current Guidelines.[45] This leads to

Conclusion 7: the current review of decisionmaking be better resourced to ensure earlier input into PBAC proceedings

Conclusion 8: the Guidelines be amended progressively rather than a complete new edition waiting until 1999.

6.3 3. Company access to the Department

The Pharmaceutical Evaluation Section (PES) in the Department of Health and Family Services undertakes detailed technical analysis of the clinical and economic issues raised in each company submission to the PBAC. These assessments are provided to ESC and PBAC. Given the technical complexity of the cost effectiveness analyses in regard to new drugs, it is clear that the PES assessments play a major role in the handling of the economic aspect of submissions by the PBAC.

[45] The case for replacing infrequent large scale updates of the Guidelines with a less ambitious but more expeditious approach has been reinforced by the landmark publication in 1996 of Gold M.R. et al, Cost Effectiveness in Health and Medicine. This is a major source book on the state of play in using cost effectiveness analysis in drug listing decisions.

In the course of the consultancy a number of PES assessments were examined in the context of the thirty case studies mentioned earlier in this Appendix.

The consultants formed the view that the quality of PES assessments of company submissions is technically very good. Of particular importance in this regard is the requirement that the company provide to the Department a floppy disk containing the data and equations used to compute the cost effectiveness information for its proposed drug. This enables the PES itself to change key assumptions made by the company and to advise the PBAC of the effect of this on the final cost effectiveness ratio.[46]

This technical competence is reflected in a generally high standard of briefing for ESC and PBAC.

There are some areas, however, where it is possible to identify improvements in practice. It is notable that PES assessments of company applications identify many uncertainties in the company application (relating to assumptions used, sources of data, adjustments made to data etc.). These uncertainties are noted for consideration by ESC rather than being resolved by what often might be a quick question to the company. The reflection of these uncertainties through to the decision making level results in a less perfect information base for ESC/PBAC decision than need be the case if the PES had the time and resources to contact the company with a query list.

This point is also related to a criticism by the companies that the drug listing process lacks transparency and that they are not asked to speak to their proposal by the PES (although they are asked to be prepared to do so by the Guidelines). PBAC

[46] However, as indicated elsewhere in the report, an improvement on existing arrangements would be for the company to meet with the Department (possibly together with independent assessors) to be quizzed on possible weaknesses in its application.

has taken steps to improve communication with the industry. However a further step could be the relatively straightforward introduction of a question and answer session between the company and PES as a standard part of the PES analysis of the each company submission.

- as well as improving the information base for decision making this would also help to reduce company angst about the 'black box' nature of the listing process as a whole
- it might also enable the Department to test the prices assumption on which the company based its cost effectiveness analysis **before** the listing proposal is considered by ESC and PBAC.[47] This leads to

Conclusion 9: there is a case for the Department inviting companies 'in-house' to speak to their more significant proposals. This would reduce the need for PES assessments to transmit uncertainties in company submissions into the ESC/PBAC committee rooms and provide companies with a more interactive role in the listing process. However it would require additional PES resourcing.

[47] The inclusion of company interviews as part of the run-up to ESC/PBAC consideration would require additional staffing of PBB. However decisions to list new drugs are effectively new policy decisions which in many cases have very large budget impacts, but which under present practices receive much less scrutiny by the government than the normal run of new policy with comparable impact on the budget.

7. C. The third link in the quality chain: the extent to which the PBAC exploits the full potential of cost effectiveness information in its listing recommendations.

The contribution made by cost effectiveness information to value for money from the PBS depends not only on the quality of the economic information sought from and provided by companies proposing new drugs but also the extent to which PBAC fully exploits the potential for this information to improve the internal consistency of its listing recommendations.

PBAC faces challenges in making full use of the wealth of economic information available to it, and in reconciling this information with the clinical issues which are its major focus. This reflects several factors, including the technical complexity of the issues it handles and the sheer size of its agenda.

One further factor is that the **membership** of the PBAC has a primarily clinical focus (as required by the National Health Act) which mitigates against fully exploiting the economic evidence which is provided to it.[48] From the consultants' perspective, in an environment in which the management of the cost of the Scheme is likely to continue to grow in importance, there is a case for adding one or more health economist to the membership of the PBAC.

A further issue relates to the way in which PBAC uses cost effectiveness information in recommending whether or not to list a drug. As indicated above, where the benefits offered by a new drug over the existing treatment (in quality adjusted life years)

[48] Currently the membership of the PBAC is determined by the National Health Act (1953) and comprises mainly clinical specialists, pharmacists and GPs, and there are no health economists.

exceed a threshold level of additional cost to the PBS, the new drug is not likely to be recommended by the PBAC. However the determination of this threshold is informal and undocumented. **While it marks a boundary between what is regarded by PBAC as acceptable value for money for improved outcomes and what is unacceptable, it is unclear how this boundary relates to the management of the overall size and cost of the PBS program.**

One way of addressing the need for the PBAC to recognise an implicit budget constraint on its recommendations is for it to consider grouping its recommendations into perhaps four categories. Category 1 would be submissions very strongly recommended for listing, ranging down to Category 4, being submissions not recommended for listing. Ministers might then make final decisions on listing in the light of this PBACs prioritisation, that is beginning with Categories 1 and 2 and moving on into Category 3 as far as budget considerations permit. This leads to

Conclusion 10: either cost effectiveness thresholds used by the PBAC be made more explicit or the PBAC focus on placing its recommendations in league table format (or groupings) to assist ultimate decisionmakers manage overall program cost.

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