Medicines Australia
Submission to the
Productivity Commission Inquiry

Impact of Advances in Medical Technology on Healthcare Expenditure in Australia

January 2005

MEDICINES Australia

BETTER HEALTH THROUGH RESEARCH AND INNOVATION

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All figures shown in this document are in Australia dollars unless specified otherwise.

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RECOMMENDATIONS AGAINST TERMS OF REFERENCE

The key drivers of medical technology demand
(See sections 5, 6, 7)

- Expand the influence of market signals in consumer choice and ensure the financial sustainability of the PBS by reforming the current system of co-payments.
- Encourage greater private funding by investigating alternative funding arrangements, such as medical savings accounts and private health insurance coverage of medicines.
- Minimise possible health risks to patients in the future by considering the phase-in of policy-induced co-payment increases over a period of time, such as one year.

Impact of advances in medical technology on healthcare expenditure
(See sections 2, 5)

- Improve the effectiveness of healthcare expenditure by ensuring that policy development to maintain the future sustainability of the PBS involves all key stakeholders, including medical practitioners, industry, pharmacists and consumers at key policy formation and decision making stages.
- Achieve greater certainty in future PBS expenditure by government and industry working together to develop forecasts of expected growth.
- Improve the financial sustainability of the PBS by reviewing the efficiency of the supply chain.
- Develop better assessment of new pharmaceutical technologies, such as 'biologicals', by government and industry working together more regularly to determine how such technologies can be best evaluated.

Mechanisms and processes for ensuring cost-effectiveness
(See sections 3, 4)

- Increase flexibility in the choice of a comparator for evaluating the cost-effectiveness of new patented medicines, how that choice is made and when less direct comparisons between medicines can be applied.
- Provide better rewards for advances in pharmaceuticals technology and intellectual property by reviewing the various price control measures.
- Protect the value of innovative, patented medicines listed on the PBS by adjusting the price of the comparator at least for inflation and by reforming the reference pricing system.
- Ensure that the contribution of innovative medicines and their impact on health expenditure is fully valued through greater PBAC acceptance of advanced methodologies of cost-effectiveness evaluation, including cost-benefit approaches, quality of life estimates and indirect costs.
- Provide better consideration of the likely impact of new medicines on health expenditure by allowing greater flexibility in the PBAC's 'hierarchy of evidence' required for PBS submissions and in the PBAC's decision making when dealing with uncertainty.

- Broaden access to innovative medicines for those patients who could benefit, by reviewing the use of restricted listings.
- Ensure that Australians fully capture the benefits of new technology by reviewing the extent to which PBS reimbursement is narrower than TGA indications and how this impacts on access to advances in technology.

Impact of changes in medical technology on the distribution of costs in health system
(See section 8)

- Increase appreciation of the potential for advances in pharmaceuticals technology to provide off-setting savings in other parts of the health system.
- Provide better rewards for advances in pharmaceuticals technology and intellectual property by reviewing the various price control measures.
- Ensure that the contribution of innovative medicines and their impact on health expenditure is fully valued through greater PBAC acceptance of advanced methodologies of cost-effectiveness evaluation, including cost-benefit approaches, quality of life estimates and indirect costs.

Impact of advances in health technologies on economic, social and health outcomes
(See section 9)

- Improve the recognition of the benefits that innovative medicines deliver to the community and the economy in government policy, estimates of future healthcare expenditure and in the PBS listing process.
- Improve the rewards for introducing advances in pharmaceuticals technology by reviewing the size of the Pharmaceutical Partnerships Program (P_3) and ensuring that the Factor (f) criterion is reflected in pricing decisions.
- Develop a Government White Paper on the National Medicines Policy, with particular focus on reviewing the role and effectiveness of medicines and the PBS in providing Australians with the latest available medicines, particularly in the context of an ageing population.
- Ensure that the contribution of innovative medicines and their impact on health expenditure is fully valued through greater PBAC acceptance of advanced methodologies of cost-effectiveness evaluation, including cost-benefit approaches, quality of life estimates and indirect costs.
- Provide better consideration of likely impact of new medicines on health expenditure by allowing greater flexibility in the PBAC's 'hierarchy of evidence' required for PBS submissions and in the PBAC's decision making when dealing with uncertainty.
- Ensure that the wider social and economic benefits of new medicines are evaluated by encouraging greater government-industry dialogue to determine how such indirect benefits of medicines can be captured in the assessment process for new PBS listings.

EXECUTIVE SUMMARY

- Pharmaceuticals are a major medical technology influencing Australia's health system and an essential pillar of national health policy.
- Technological advances are giving rise to a whole new generation of medicines to cure major diseases.
- The challenge is to ensure that Australia has a system in place that provides Australians with timely access to new medicines into the future while ensuring the system is sustainable from a fiscal, health and industry policy perspective.
- Australia's system of ensuring access to pharmaceuticals is a complex mix of evaluation, subsidy, pricing and reimbursement where the price Australia pays for new, high technology medicines is low by international standards.
- Pharmaceuticals are likely to continue to grow as an area of health expenditure in Australia. However, the overall pattern of growth needs to be better understood, defined and appreciated.
- While the subsidised nature of the PBS makes the demand for new pharmaceutical technology somewhat inelastic in Australia, this is changing as patient co-payments evolve.
- The demand for innovative medicines is driven by a number of factors, not least being Australia's National Health Priority Areas and the emerging evidence supporting the use of these medicines.
- Expenditure on pharmaceuticals, particularly newer, high technology pharmaceuticals, can be demonstrated in many instances to be accompanied by substantial and real cost-offsets within other areas of the health system. These deserve greater recognition in policy and HTA processes than is currently the case.
- Spending on new, innovative medicines provides broader economic and societal benefits such as enhanced quality of life, increased productivity and workforce participation. This is not always sufficiently recognised.
- The growth in health expenditure on pharmaceuticals in Australia should be viewed more favourably given Australia's relative level of spending, international trends, and the benefits that accrue to the health system and the broader community.
- The process of HTA as applied to pharmaceuticals in Australia has significant impact on the pharmaceuticals industry, with outcomes that often include world-low prices, delays in reimbursement, delays in Australians' access to new medicines and reduced effective patent life.
- The actual process of listing a new, innovative medicine in Australia under the PBAC guidelines is extensive, complex and uses theoretical standards that are often difficult to achieve in terms of global pharmaceutical research and development. Improvements are being made to the operation of PBS-related processes as a result of the US-Australia Free Trade Agreement.

1. INTRODUCTION

"The most important factor differentiating the practice of medicine in 1999 from that in 1899 or 1949 may well be the availability of increasingly powerful and effective drugs, such as antibiotics, cancer controlling drugs and thrombolytics, to mention just a few"[1].

Medicines have played a pivotal role in improving the health of humanity. Technological improvements in medicines have led to increased life expectancy, improved quality of life, increased productivity, enhanced workforce participation and made a more efficient health system. Medicines have eliminated diseases that in times past were major threats to human health.

The challenge, now and into the future, is to ensure that Australians have access to the latest medicines available. This is likely to become particularly important with the ageing of the population. Medicines have a key role to play in improving the health of Australians in the future. Growing health expenditure as result of advances in medical technology, such as in new, innovative pharmaceuticals, may not be detrimental given the range of benefits for health and broader society from that technology. The issue may be in adequately recognising and rewarding such improvements in medical technology and ensuring that Australians will gain access to them into the future.

A number of factors will help to ensure that Australians will have access to the new medicines being developed. Reforming the process of evaluating the cost-effectiveness of medicines will improve health technology assessment and the availability of new medicines. Ensuring the long-term sustainability of the Pharmaceutical Benefits Scheme (PBS) – both financial and health outcome sustainability – will provide certainty of future access for patients. Understanding the drivers of demand for new medicines and new technology will facilitate the better management of future demand and health expenditure. Allocating sufficient resources to the PBS will ensure Australians can access to new medicines as they become available. A better understanding of the role of the PBS in delivering sustainable health outcomes and its benefits for the health system, the economy and society will allow more informed decisions to be made about accessing new pharmaceutical technology.

Medicines Australia welcomes the opportunity provided by the Productivity Commission to make a formal submission to its review, *The Impact of Advances in Medical Technology on Healthcare Expenditure in Australia*. This submission focuses on the application of the terms of reference to the case of innovative pharmaceuticals. These represent technology that is continuously evolving, subject to very high research and development costs and complex risk assessment processes. The Federal Government, through the Pharmaceutical Benefits Scheme, spends around 14 per cent of Commonwealth health expenditure on pharmaceuticals.

[1] Jacobzone, S. 2000 *Pharmaceutical Policies in OECD Countries: Reconciling Social and Industrial Goals*, Labour Market and Social Policy Occasional Papers, No. 40, OECD: Paris, p. 9.

While almost all countries have systems for the evaluation of safety and efficacy of pharmaceuticals, only a few have specific health technology assessment (HTA) policies and processes. Not surprisingly, these are more sophisticated in countries with national public health systems and subsidy of pharmaceuticals. Arguably, Australia leads the field in terms of its history with and complexity of HTA as applied to pharmaceuticals.

Australia's system of assessing and funding innovative medicines needs a range of reforms to ensure that Australians continue to have access to the range of innovative medicines that are available now, as well as new medicines currently in the development pipeline. This submission examines these issues and how they affect the impact of advances in medical technology on healthcare expenditure in Australia.

1.1 Structure of this submission

This submission argues that further progress needs to be made at a technical level to ensure that the benefits to Australia from advances in pharmaceutical technology are realised. Each section of the submission discusses how advances in a major form of medical technology, innovative medicines, are assessed and utilised in Australia.

Section 2 is an introductory overview of the PBS funding mechanism and decision processes. It provides some background on the current system and how it operates in the broader health sector, with some comments on the processes of HTA as they relate to medicines which are expanded further in the submission.

Section 3 provides a more detailed discussion of the actual process that is used to assess new pharmaceutical technologies in Australia. It discusses the system that pharmaceutical companies work with to have a new medicine listed on the PBS and identifies where the current system does not sufficiently value innovative medicines and adversely impacts on companies' ability to bring new medical treatments to the Australian population.

Section 4 discusses the impact of these processes on Australians' access to new medicines. It highlights the need for the current system to appropriately reward new, innovative treatments and the implications of where this does not occur, both globally as well as for Australians.

Section 5 examines the PBS and the likely trends impacting on future expenditure on medicines in Australia. This section looks at PBS spending to date, whether this is appropriate, the scheme's financial sustainability, and future trends. It also examines the impact of older medicines going off patent and new medicines being developed. This section canvasses options to ensure that Australians can have timely access to the latest innovative medicines into the future, including options for PBS co-payments.

Section 6 analyses how the use of medicines is affected by co-payment increases which are important to ensuring the future financial sustainability of the PBS. It discusses international and Australian research on the impact of co-payment increases on the use of medicines and flags strategies to avoid the short-term effects of co-payment increases on the quality use of medicines.

Section 7 looks at how other non-price drivers that influence demand for new, innovative medicines. Factors examined here include government policy, prescriber behaviour, consumer expectations, industry promotion, and patient information. These factors affect health expenditure and illustrate how expenditure on innovative medicines meets the health needs of Australians.

Section 8 examines international and Australian evidence that medicines provide offsetting savings in other parts of the health system. It reviews international literature and whether Australia's level of spending on pharmaceuticals should be a concern. This section also provides several detailed case studies on how innovative medicines have offset other health costs, reducing the pressure in other areas of health expenditure.

Section 9 examines the wider benefits of innovative new medicines in terms of their impact on social and economic outcomes. The growing body of international and Australian evidence on the economic and social benefits of medicines is reviewed. The extent to which these benefits are considered in the current Australian process of listing new medicines is also examined.

Section 10 provides some concluding remarks as well as some suggestions on ways forward to reform and improve the current system so that Australians will have timely access to the latest, innovative new medicines as they become available.

2. OVERVIEW OF PBS FUNDING MECHANISMS AND SUBSIDY DECISION PROCESSES IN AUSTRALIA

Advances in pharmaceutical technology and their adoption in Australia are influenced by the broader health, fiscal and industry policy environment. The system of delivering medicines to Australians, the ongoing management of the operation and cost of that system, its interaction with the wider health sector, and the incentives for developing new medicines all interact to influence the uptake of advances in pharmaceutical technology. This section provides an overview of this system, flagging some of the issues that are expanded on further later in the submission.

Federal Government funding of prescription medicine costs is administered through the Pharmaceutical Benefits Scheme (PBS), a comprehensive centralised formulary listing reimbursable products. The Pharmaceutical Benefits Branch of the Department of Health and Ageing (DoHA), along with the Pharmaceutical Benefits Advisory Committee (PBAC), administers the scheme. The PBAC is a statutory body that makes recommendations on product listings to the Minister for Health and Ageing, based on an assessment of the cost and effectiveness of a medicine. This requires submission and assessment of economic evaluations of the medicine in question.

Overall the PBS (government and patient contributions) accounts for around 90 per cent of total prescription of pharmaceutical expenditure. Government spending on prescription medicines under the PBS rose by 9.3 per cent in 2003-04 to $5 billion. Patient contributions added a further $938 million, thereby taking total prescription medicine costs to $5.9 billion. The number of prescriptions dispensed under the PBS grew in 2003-04 by 4.3 per cent to 156 million. Processing and payment of PBS claims to pharmacists is the responsibility of the Health Insurance Commission (HIC).

The prescribing of new, higher-priced, products is one of the main components driving PBS expenditure.

In 2003-04, the therapeutic categories placing the greatest financial burden on the PBS were:

- Cardiovascular medicines ($1.89 billion);
- Nervous system agents ($1.07 billion);
- Alimentary tract/metabolic products ($870 million); and
- Musculo-skeletal products ($435 million)[2].

The four most costly products reimbursed under the PBS in 2003-04 were:

- Atorvastatin ($427 million). This is a medicine in the ‘statin’ group that lowers cholesterol and improves the balance of various lipids in the body, thereby reducing the risk of cardiovascular disease;
- Simvastatin ($373 million), another a medicine in the ‘statin’ group;

[2] DoHA 2004 *Expenditure and prescriptions twelve months to 30 June 2004*, Pharmaceutical Pricing Section: Canberra, p. 7.

- Omeprazole ($209 million). This is a proton pump inhibitor, which suppresses the production of acid in the gastric system, thereby assisting healing of duodenal and gastric ulcers, or reducing the symptoms of gastro-oesophageal reflux; and
- Salmeterol/fluticasone ($177 million). This is a combination therapy for asthma[3].

The substances generating the most prescriptions under the PBS during 2003-04 were:

- Atorvastatin (6.6 million prescriptions, costing $427 million);
- Simvastatin (5.5 million prescriptions, costing $373 million); and
- Paracetamol (4.1 million prescriptions, costing $32 million)[4].

Each of these medicines represents an advance in medical technology and each of these, except for the statins, are more expensive than the previous technology that they replace. Where the products are more expensive they will have justified their higher price through the process of economic evaluation. For example, the statins (represented here by simvastatin and atorvastatin) have generally replaced bile acid sequestrants in the treatment of hypercholesterolaemia. On current prices there is no difference between the two classes although the statins are more convenient to take and more tolerable which partly explains the significantly higher usage of the statins relative to the bile acid sequestrants.

The Government has introduced a range of measures in place that control PBS spending. Prominent among these are:

- Restricted and 'Authority required' listings;
- Restrictive pricing policies;
- Imposition of cost effectiveness requirements;
- Generic substitution;
- 'Quality use' initiatives; and
- Patient co-payments.

PBS listing policies are discussed below in general terms and in detail later in this submission.

2.1 PBS listing

While most prescription medicines are listed on the PBS, many are not reimbursed freely, and even when reimbursed, this is usually much narrower than the indications for which medicines are approved by the Therapeutic Goods Administration. Three listing categories define the conditions attached to reimbursement of individual medicines. These are:

- ***General listing***: Reimbursement applies to all prescribed indications. This typically applies to older, low-cost medicines, such as early-generation antibiotics;

[3] DoHA 2004 *Expenditure and prescriptions twelve months to 30 June 2004*, Pharmaceutical Pricing Section: Canberra, p. 21.
[4] DoHA 2004 *Expenditure and prescriptions twelve months to 30 June 2004*, Pharmaceutical Pricing Section: Canberra, p. 23.

- ***Restricted listing***: Reimbursement is offered for indications where the product has demonstrated cost-effectiveness (see Cost Effectiveness Requirements). For example, Losec (omeprazole) is reimbursable when prescribed for reflux oesophagitis, but not for the treatment of milder gastric conditions; and
- ***Authority required listing***: Reimbursement is restricted to a specific disease classification, often with associated eligibility criteria. Doctors must obtain prior approval from the HIC before completing prescriptions for medicines in this category.

An independent analysis by the Centre for Strategic Economic Studies found that almost two thirds of items listed on the PBS in 2002 had some form of restriction (Table 1).

Table 1: Restrictions on PBS items

	Number of Items	Cost in 2000-01	
		$m	%
Authority required	500	1,092.4	24.5
Restricted benefit	669	1,913.0	42.9
No restriction	1,134	1,449.0	32.5
TOTAL	2,303	4,454.5	100.0

Source: Sweeney, K. 2002 *Trends in the Use and Cost of Pharmaceuticals under the PBS*, Working Paper 5, Centre for Strategic Economic Studies, Victoria University of Technology: Melbourne.

Restricting the listing of a medicine is one strategy that is increasingly being used as a means of controlling the cost of PBS reimbursement to the Government. Companies are also required to demonstrate that new products are cost effective in comparison with established medicines or therapeutic regimes. Sections 3 and 4 discuss the listing process in more detail and its impact on the availability of new pharmaceutical technologies. An extensive discussion of restrictions is contained in Section 4.

2.2 Hospital sector

There are around 730 public hospitals in Australia, with a total bed capacity of close to 57,000. Hospital funding is provided from the Federal Government's healthcare budget and the GST payments to the States, but responsibility for managing the public hospital infrastructure lies with State administrations.

Pressure on the public hospital sector has increased as a result of declines in private health insurance and the impact of high-technology procedures. Funding and staffing shortages are widespread in the system. This has prompted recent increases in hospital waiting lists, and has forced hospitals to focus more closely on spending. Some in the public hospital sector have suggested that the failure

to address the waiting list problem is part of a broader strategy, designed to push the patient population back into the private sector.

The PBS provides limited coverage of public hospital medicine costs. Under recent reforms, some States have agreed to PBS cover of discharge prescriptions dispensed at hospital pharmacies in exchange for efforts related to continuity of care. In-patient use of pharmaceuticals is funded directly from individual hospital budgets. Patients receiving treatment in private hospitals, however, can access the scheme.

In addition to medicines listed under the PBS, a number of 'Highly Specialised Medicines' are listed under Section 100 in the PBS schedule. These medicines are for the treatment of highly specialised, usually chronic conditions, such as HIV/AIDS, rheumatoid arthritis and hepatitis C and B. They are often administered at special clinics and are subsidised by the Commonwealth Government.

Cost shifting to the primary care sector is a means for hospitals to cut pharmaceutical costs. Earlier patient discharges also reduce hospital pharmacy costs, since primary care prescription costs fall under the remit of the PBS. Other efforts by public hospitals to reduce pharmacy spending include the use of formularies, increased generic prescribing and purchase tenders. Medicine formularies are less restrictive than those operated in many other countries.

One issue that has not received sufficient attention to date is the potential for advances in pharmaceutical technology to provide savings to other parts of the health system, such as reduced use of hospital services. This is discussed in more detail in Section 8.

2.3 Private health insurance

The proportion of the Australian population covered by private health insurance declined dramatically from the late 1980s, from almost 50 per cent to a level of about 30 per cent in the late 1990s but has risen again to around 45 per cent as a result of various government incentive programs. Employee health insurance schemes are relatively rare, and most private insurance is on an individual basis. Most people with private insurance have both hospital and ancillary cover. Pharmaceuticals coverage by private health insurance is generally limited in Australia. The scope for the private sector to play a greater role in funding medicines in Australia is discussed in Section 5.

2.4 Pricing system

Pharmaceutical price control is an established focus of the Federal Government's efforts to control PBS spending. This approach to pricing has had the effect of pushing prices down below average levels of other leading industrialised markets, over time reducing the real value of the reward paid for the development of new medicines. The current system of price control is likely to remain a priority issue into the future, particularly with an increased focus on the cross national price differentials as a result of the impact of the Internet on cross border

purchasing, greater public awareness of price differentials and growing use of economic evaluation.

Significant changes, introduced during the 1990s, have had significant impacts on medicines prices:

- Cost effectiveness comparisons have been mandatory since 1992 for new products listed on the PBS, and are being applied retrospectively to older products. This has had the effect of reducing price differentials for many new products in the initial period of introduction;
- The subsequent application of reference pricing through methods such as weighted average monthly treatment costs across groups. This creates a pricing link between the price of off-patent products and patented products so that the post-patent price reduction of a product can be extended to patent protected products, and yet remain within the bounds of TRIPs[5]; and
- The therapeutic group premiums (TGP) reference pricing policy introduced in four key therapeutic areas during 1998. This is an extension of reference pricing that allows premiums to be applied to a reference priced product.

Pricing issues, and their impact on Australians' access to innovative medicines, are discussed in greater detail in Section 4.

2.5 Process of PBS listing

Having gained marketing approval for a new product from the Therapeutic Goods Administration (TGA), companies submit a request for listing on the PBS, which must be supported by cost effectiveness data. Requests are submitted to the Pharmaceutical Benefits Advisory Committee (PBAC), which then makes a recommendation on listing to the Minister of Health and Ageing, subject to agreement on the price of the new product.

The PBAC is required by legislation to consider both the effectiveness and cost of therapy in making its recommendations. Since 1992, recommendations by the PBAC to list an item on the PBS are based on an assessment of whether the medicine is an effective complement to existing items on the PBS and is cost effective.[6]

For the past two years PBAC recommendations have been made public on the PBAC web site. Only limited details of recommendations are posted on the site. Arising out of the United States-Australia free trade agreement, there will be greater levels of transparency over PBS procedures and information about the recommendations it makes.

According to Professor Lloyd Sansom, Chair of the PBAC, while 'comparative cost effectiveness forms the basis of [a] decision', other factors are taken into consideration including:

[5] Agreement on Trade-related Aspects of Intellectual Property Rights.
[6] Stephen J Duckett, 2004, "Drug Policy Down Under, Australia's PBS", *Health Care Financing Review*, Vol 25, No.3, p 59.

- The severity of the condition being treated;
- The ability to target therapy to those likely to benefit most;
- The presence of effective alternatives; and
- The financial implications for the PBS.[7]

Negotiations on new product prices are conducted between manufacturers and the Pharmaceutical Benefits Pricing Authority (PBPA). The PBPA guidelines recommend that nine factors be considered in recommending new prices and reviewing existing prices. The first, and most dominant, is the advice provided by the PBAC on clinical and cost effectiveness. Other factors include:

- Prices of alternative brands;
- Comparative prices of medicines in the same therapeutic group;
- Cost data information;
- Prescription volume, economies of scale, expiry dating, storage requirements etc;
- Level of activity being undertaken by the company in Australia including R&D activities; and
- Overseas prices[8].

The PBPA notes that 'new medicines are most commonly recommended by the PBAC on the basis of cost-minimisation or acceptable incremental cost effectiveness ratios'.

The PBPA uses several mechanisms "to contain the price of products listed on the PBS" [9] including:

- The therapeutic group premium (TGP) policy; and
- Price volume arrangements.

New products expected to incur significant PBS costs (more than $10 million in any of the first 5 years of listing), having been recommended by the PBAC and the price having been negotiated by the PBPA, are also subject to approval by Federal Cabinet. The Department of Finance and Administration may also at times impose additional demands for price-volume agreements where concern exists over 'leakage' (where the PBS subsidy is paid for an indication which is prescribed by a medical practitioner and is outside the PBS indication – see Section 7). Delays can also sometimes occur in listing a new products during price negotiations with the PBPA. The recently completed 'Post PBAC Review' produced a series of recommendations to improve on the timeliness and processes required to list a product on the PBS after the PBAC makes a recommendation. While these measures were introduced to help manage healthcare expenditure, they can delay or unduly limit Australians access to new pharmaceutical technologies.

[7] Lloyd Sansom, 2004, "The subsidy of pharmaceuticals in Australia: processes and challenges", *Australian Healthcare Review,* vol 28, No.2, p198.
[8] Pharmaceutical Benefits Pricing Authority, *Procedures and Methods*, November 2003, viewed 25/11/04.
[9] Sansom, p200.

2.6 Cost effectiveness data

Proof of the 'cost effectiveness' of a new medicine is a key requirement of PBS listing. New products must be shown to be cost effective in relation to both products already listed and alternative treatment regimes. Stringent cost effectiveness requirements have been compulsory for new products since 1992, and are being applied retrospectively by the PBAC to older products wherever possible. By 1998, cost effectiveness reviews had been applied to 31 per cent of all products listed on the PBS.

One of the industry's concerns about cost effectiveness reviews is the choice of 'comparator' products against which new medicines are gauged. A comparator is the alternative therapy used as a basis for comparison when a new medicine is being considered for listing on the PBS. While a new product may offer significant advances over existing therapies, its cost effectiveness is very often judged in comparison with an established, often generic product, which is listed at a lower price. As a result, companies often find it difficult to demonstrate the value of new medicines and technologies in the context of Australian healthcare expenditure. Section 3 provides more discussion on the choice of comparator and how this impacts on the availability of innovative medicines.

Guidelines on the presentation and assessment of cost effectiveness data have been updated periodically since 1992. A further review of the guidelines is currently under way which could be completed in 2005. The industry is seeking more flexibility in the guidelines, particularly with respect to the selection of the comparator product, and the methodology including the treatment of indirect costs and use of monthly treatment costs. A range of other issues covered in this submission, such as recognition of broader social and economic benefits of new medicines, the potential for less invasive treatments that provide savings in other parts of the health system could be considered in the guidelines review.

2.7 Brand premiums and therapeutic group premiums

Where a generic product exists in a therapeutic class, the subsidy level will be set at the lowest generic price. Manufacturers of off-patent products are permitted to apply a brand price premium to their off-patent products that face competition from generic medicines listed on the PBS. Patients who wish to use the original branded product, rather than bioequivalent generics, pay price premiums. While innovator companies are free to determine price premium levels, most apply relatively modest increases in the knowledge that patients will otherwise switch to cheaper generic equivalents.

In 1997, the Federal Government announced its intention to introduce another price control policy, the Therapeutic Group Premium (TGP) pricing system. Originally slated for application to six therapeutic groups, it was intended to generate PBS savings of $560 million over a four-year period. The Government committed $4 million to a public education campaign undertaken by pharmacists following the introduction of this system.

Two of the six product groups (Beta-blockers and Selective Serotonin Reuptake Inhibitors - SSRIs) were omitted from the TGP system when it was introduced in

February 1998 after strong lobbying from the industry. The groups subject to the TGP policy were:

- Statins;
- Calcium channel blockers;
- Ace inhibitors; and
- H2 receptor antagonists.

TGPs are essentially a form of reference pricing. Reimbursement under the PBS is reduced to the level of the lowest-priced product (usually a generic) in each of the affected therapeutic groups. Manufacturers of other products in the group are free to apply a 'therapeutic group premium' to the price of their product, but patients must pay the difference between the PBS reimbursement ceiling and the 'premium' price. Technically, a mechanism exists under which doctors can obtain authority for individual patients to remain on a particular therapy without losing reimbursement status. The process, however, is complicated and is not widely deployed.

The PBPA calculates reference-price 'benchmarks' in TGP groups by comparing the monthly treatment costs of medicines in a particular therapeutic group. The imposition of reimbursement ceilings at these price benchmarks has had a significant effect on prices in the four therapeutic areas to which it has been applied. Prices of more than half of all branded products affected by the system have been reduced to the reimbursement 'benchmark'. About a quarter of the remaining products now carry a therapeutic premium ranging from 70 cents to $4.50, but companies are loath to levy significant premiums since, by doing so, they risk dramatic reductions in market share.

These processes and their interaction with the system of reference pricing are examples of where mechanisms to manage the growth in healthcare expenditure limit the extent to which advances in medical technology are provided sufficient incentive. The system of reference pricing is covered further in Section 3.

2.8 Prescribing behaviour

The decisions that doctors make with their patients about the appropriate forms of medication are an important influence on the demand for new technology and subsequent levels of healthcare expenditure. For example, prescribing outside PBS indication can lead to higher than expected expenditure on a particular medicine. Generic prescribing is not widespread, while doctors naturally want their patients to have access to the most appropriate medicine that can cure a patient's illness. There are a range of programs and initiatives that have been designed to influence prescribing behaviour. These are discussed in more detail in Section 7.

2.9 Co-payments

One major factor that affects future healthcare expenditure, through both additional funding as well as influencing the demand for medicines is patient co-payments. Co-payments for medicines are well established in Australia. Co-payment levels are linked to movements in the consumer price index, but have also been increased at regular intervals by the Government during the past

decade. Co-payments towards the cost of prescription medicines on the PBS take the form of prescription fees and on 1 January 2005 were levied at:

- $28.60 per prescription for the general public; and
- $4.60 per prescription for concession card holders (pensioners, the unemployed and other welfare recipients).

The maximum payment or 'safety net' threshold for the general public is $874.90 per annum and $239.20, or 52 scripts, a year for concession card holders. Concession cardholders account for around 80 per cent of all PBS prescriptions. PBS co-payments increased on 1 January 2005 with general patients' co-payments increasing from $23.70 to the current $28.60, and concession cardholders' co-payments increasing from $3.80 to $4.60.

It should also be remembered that patients who wish to take an original branded product rather than a cheaper, bioequivalent generic must pay the relevant price premium, or the therapeutic group premium (TGP) for products affected by this system in addition to the co-payment.

Medicines Australia supports the co-payment increases and the development of a responsible co-payment system, provided that the health and wellbeing of those that can least afford the increases is not compromised. Co-payments help to ensure the future financial sustainability of the PBS. Discussion of the potential for further reform of co-payments is discussed in Section 5 in the context of future PBS sustainability and the range of new high technology medicines being developed now. Section 6 discusses how co-payments impact on the use of medicines and potential implications of increases for the quality use of medicines.

2.10 Patents

Patent terms for innovative pharmaceuticals in Australia were harmonised at 20 years under amendments to the country's Intellectual Property Law, which came into effect in 1996. However, the Intellectual Property Law Amendment Bill of 1998 superseded this legislation (with effect from July 1999). Essentially, the new law brings Australian patent protection into line with exclusivity provisions in the USA, Europe and Japan. Products with patents in force on or after 1 July 1999 are eligible for a maximum five-year extension period, designed to compensate for time spent in the product approval process and to deliver an effective patent life of 15 years from date of marketing approval. Applications for patent extension in respect of eligible products must be submitted within six months of marketing approval in Australia.

Improvements in patent protection are perceived by the Federal Government to be a necessary trade-off in order to secure continued investment in Australia by the R&D based pharmaceutical industry and encourage advances in pharmaceutical technology. While innovator companies welcomed these improvements, the system of reference pricing, and its interaction with brand and therapeutic premiums, substantially devalues patent protection by reducing returns for new products while they are still under patent. This effectively reduces the significance of patent protection and is discussed further in Section 4.

2.11 Research and Development

The impact of the PBS on prices in Australia is therefore a significant factor influencing pharmaceutical R&D and investment decisions within the industry. In the past, the low prices paid for innovative medicines in Australia have been partially offset by significant industry incentives designed to encourage R&D and investment as there was recognition that low prices were a disincentive to innovation.

Annual investment in research and development by the pharmaceutical industry in Australia is around $450 million, up from $227 million in 1997. The industry is an important partner for institutional research organisations in Australia, contributing an estimated 15 per cent to total research budgets in this sector. Around 90 per cent of pharmaceutical companies based in Australia are involved in R&D. Between them, they collaborate in more than 260 alliances with research centres in the country.

Since the late 1980s, the Federal Government has offered incentives for the pharmaceutical industry to undertake R&D or value-added manufacturing activity in the country. These initiatives amount to a trade-off, designed to encourage industry investment in Australia in spite of the relatively poor pricing environment that exists here.

Under the so-called Factor (f) scheme, which ran in two phases from 1988 to 1999, participating companies were granted notional price increases for PBS listed products in return for increases in R&D or production activity. Government expenditure on the Factor (f) scheme totalled approximately $1 billion. The Factor (f) scheme was pivotal in retaining the Australian pharmaceutical industry and securing new investment and R&D in the face of global rationalisation during the 1980s and 1990s.

Guidelines for the subsequent scheme – the Pharmaceutical Industry Investment Programme (PIIP) – were circulated in 1998, and the new programme commenced on 1 July 1999. A total of $300 million was available to participating companies over a five-year period, in return for commitments to increase investment, research and production value-added (PVA) activities. This was a far smaller sum than that allocated under the Factor (f) scheme. Consequently, while a total of 17 companies were involved in the Factor (f) programme, only nine participated in the PIIP. (There were 20 applications but only nine were successful.) The limited scope of the PIIP has been a focus of industry concern.

The nine companies participating in the PIIP and their commitments are detailed in Table 2. Between them, they planned to generate at least $1.5 billion in additional activity as well as increasing employment in the industry by more than 1,000 people. The total value of their activities over the five-year period was forecast to be $6 billion.

Table 2: Outline of Remuneration and Commitments Under the PIIP

Company	PIIP Funding $ (million)	Commitments
AMRAD	20	$ 120m R&D spend; $ 228m PVA
BMS	39	Double R&D spend; $ 155m PVA
CSL	60	$ 300m increase in R&D and PVA
Lilly	20	$ 100m increase in R&D
F H Faulding	40	Unspecified increases in R&D and PVA
Glaxo Wellcome	27	$ 137m increase in R&D and PVA
Janssen-Cilag	18	$ 87m increase in R&D and PVA
Pfizer	39	$ 194m increase in R&D and PVA
P&U	34	$ 169m increase in R&D and PVA

Source: Department of Industry, Science and Resources.

Australia is a relatively inexpensive location for multinational companies to conduct research. The country also boasts a large supply of skilled employees and a sound institutional research base, but it is being forced to compete for research investment from an industry that is undergoing significant rationalisation on a global scale. With the growth in financial incentives around the world, it is important to prevent the erosion of R&D and investment activity by companies in Australia. The relatively low prices available to pharmaceutical companies in Australia will continue to have an adverse effect on investment and continues to provide the basis for additional government support through investment incentives to industry.

The current Pharmaceuticals Partnerships Program (P_3) is the first step in a new Government industry development plan to implement the Action Agenda. P_3 is relatively small compared with the former Pharmaceutical Industry Investment Program (PIIP) and Factor (f) programs. While P_3 is a very welcome first step, there is a need to review its size. At $150 million over five years, with a $10 million cap per applicant over the life of the program, this is a significantly lower level incentive than earlier programs.

P_3 is an important building block but the major rationale for industry development in this sector – the effect of pricing on activity – is explicitly not a feature of P_3 as it was for earlier industry programs. A good starting point would be a review of the Pharmaceutical Benefits Pricing Authority pricing guidelines to ensure that the Factor (f) criterion is reflected in pricing decisions.

3. PROCESS OF HEALTH TECHNOLOGY ASSESSMENT FOR MEDICINES IN AUSTRALIA

"Health technology assessment (HTA) can encourage innovation if assessments are properly done and consider a wide range of costs and benefits associated with a new technology rather than simply focus on acquisition costs. The expense must be viewed in terms of the broader benefits that would arise if the technology were adopted. This may require that our governments allow expenditure levels to be driven by value and not arbitrary budget caps." [10]

Advances in health technology, particularly the development of innovative medicines, provide a wealth of benefits for the individual, the health system, the economy and the broader community. Once diseases and illnesses can be treated with innovative medicines, existing and often more costly treatments are replaced by these less costly and less invasive treatments, allowing individuals to actively participate in their community again.

The ability to demonstrate the value of new technologies, such as innovative medicines is critical and facilitates a more objective way of assessing an appropriate level of expenditure on these technologies.

Such assessment relies on many factors including the availability of appropriate information about the value of innovation and the extent to which the system recognises that this information is available. In Australia, the requirements for demonstrating the value of new medicines, differ from those in most other countries are hampered by a lack of reliable local information and are considered by many to be overly restrictive. This can makes it very difficult to demonstrate the full value of new pharmaceutical technologies and therefore to justify and advocate an appropriate level of expenditure.

3.1 Australia's restrictive requirements in demonstrating value

The requirements under the PBS system for listing a medicine remain one of the most vigorous of all reimbursement systems in the world. The guidelines for applications for a PBS listing require a level of clinical evidence and cost effectiveness that is unparalleled world wide. As a PBS listing is essential for market access – there is limited prescribing in the private market outside the PBS in Australia – this directly influences universal access to medicines for Australians.

These stringent requirements also have significant resource implications for local pharmaceutical companies who seek to list a medicine on the PBS: resources in excess of what would otherwise be the case.

While the industry supports the core principle of evidence-based listing of a medicine on the PBS, there are conceptual flaws in the manner in which this principle is made operational, which contributes to an uncertain operating

[10] Professor Michael Drummond, "Promoting Innovation: does HTA help and what are its limitations", *The Impact of Health Technology Assessment on the innovative process: sail or anchor?*, Report from a pre-conference symposium, 21 June 2003, Canmore Alberta.

environment for the pharmaceutical industry in Australia. This has resulted increasingly in delayed or limited access to the latest and best medicines (see Section 4 for details on the impact and outcomes of this approach).

The implementation of evidence-based listing methodologies, governed by the PBAC, remains a concern to industry as they are associated with discouraging the listing of new agents as a pharmaceutical benefit. Recent policy initiatives developed as a result of the US-Australia Free Trade Agreement may reduce some of these concerns.

The following section focuses on the application of cost effectiveness analysis through the PBAC and areas where reforms can be made to improve the process of health technology assessment in Australia.

3.2 Introduction of cost effectiveness requirements

When Indiana University health economist Deborah Freund visited Australia in the late 1980s, she expected to give advice on the broad use of health economics approaches to a range of health issues. She was surprised when that advice specifically translated into a formal set of guidelines for the economic evaluation of pharmaceuticals for the purpose of recommendations on reimbursement. Legislation supporting the cost effectiveness requirement was passed in 1987.

The introduction of the guidelines in 1992 and the associated processes applying to the consideration of submissions by the PBAC was a world first in health care policy and practice. As a result, there was a steep learning curve for all involved and, over time, the quality of submissions and economic evaluations gradually improved.

In the early 1990s, key principles underpinning the PBAC process were:

- Strong focus on the quality of the clinical evidence supporting claims of superiority;
- Guidance on appropriate economic evaluation methodologies (without being prescriptive);
- Inclusion of, but no emphasis on, estimates of utilisation once reimbursed
- Cross functional PBAC membership, strong evaluation capacity within the PBB;
- PBAC advice based on cost-effectiveness and clinical need, with pricing being negotiated by second body, PBPA; and
- Limited interaction between sponsor companies and the process.

3.3 Major developments and changes

In the latter part of the 1990s and early part of this decade, there were some significant developments which tightened the mandate of the PBAC.

Since the introduction of cost effectiveness, one of the most significant developments in relation to the PBAC process occurred with the decision not to reimburse sildenafil. This has had a significant impact on the PBAC's approach and its scope of consideration. Although the PBAC changed its recommendation

in favour of sildenafil after an appeal in 2000, the right of PBAC to consider and comment on possible total government outlays (if a product was to be reimbursed) was enshrined. The guiding principle for the PBAC became 'value for money' for the PBS rather than value for money in the health system.

In 2001-02, the listing of two products (Celebrex and Zyban) contributed to a rapid growth in PBS expenditure. In spite of industry claims that this would be a temporary phenomenon, the central agencies of Government increased their focus on PBS outlays. The increased emphasis on predictability and fiscal risk management of PBS outlays drove several significant process changes including:

- Increased role for the Drug Utilisation Sub-Committee (DUSC) and increased scrutiny of sponsor estimates of utilisation;
- Increased use of complex restrictions for listing under the Authority system and enforcement of these;
- Lower threshold for Cabinet approval; and
- Experiments in price-volume agreements.

3.4 Reference Pricing

In Australia, the combination of the application of health technology assessment and reference pricing results in effective downward pressure on pharmaceutical pricing.

If it is difficult to demonstrate incremental value, due to methodological or other reasons, then it follows that listing is not likely to result in incremental prices or perhaps no listing at all. For cost containment purposes, the system provides a strong incentive not to agree to incremental value as this may translate to incremental cost.

This process ignores patent protection that may exist on any of the products within the class. Indeed, in some classes where reference pricing occurs, all products in the class remain in patent. As a subset of this reference pricing, the PBAC and Government have agreed that some classes are also therapeutically interchangeable on a per patient basis and thus fall subject to the Therapeutic Group Premium policy. Under this policy, a patient may be switched from one molecule within the class to another seemingly without therapeutic consequence.

The industry has identified several key areas of concerns with the methodologies and approaches used for listing items on the PBS. These are described below.

3.5 Choice of comparator

In a truly solution-oriented system, one that strives to deliver products to patients, flexibility in the choice of comparator may be of significant value. Current guidelines constrain the choice of comparator. This is a key issue because, increasingly, clinical trials are conducted on a global basis. Worldwide clinical data cannot hope to cover all potential comparators and companies can be disadvantaged by different patterns of world use compared with Australian use. Moreover, the reality of available clinical data sets, changes in clinical practice over time and differences in global treatment practices mean that, in making a PBS submission, an applicant may be constrained in demonstrating superior

effectiveness. Submissions that are deemed to adopt an inappropriate comparator may be rejected by the PBAC.

Disagreement with the PBAC over the choice of comparator is one of the major reasons cited by the industry for unsuccessful or delayed PBS submissions. Recent research has identified the major disagreements range from the choice of comparator made in the evaluation of the company's submission to the age of the comparator, particularly when the use of older, generic comparators are used to evaluate newer, innovative technologies[11]. The survey also found further difficulties in agreeing on the appropriate comparator.

"Confusion could arise when current comparator usage may not reflect approved usage, or doses were not accepted. Companies often received conflicting advice from the PBAC on the appropriate comparator to use over the course of a submission and subsequent resubmission. Also, opinion over the most appropriate comparator could differ between the PBAC and the PBPA"[12].

Where clinical data is not available to compare a new agent to the chosen comparator, indirect comparisons may be employed, but these are less likely to succeed.

Difficulties around choice of comparator are not a new issue but they need to be addressed. The industry seeks greater flexibility:

- In the choice of comparator;
- Frameworks for choosing the comparator based on both indication and clinical positioning of the proposed medicine; and
- For circumstances where indirect comparisons can be applied.

3.6 Process concerns

A related issue with comparators is the inequity in the process, due to the fact that it is easier in some therapeutic areas to demonstrate differences than in others. In therapeutic areas where there has been little progress in recent times, the comparator is often an old, out-of-patent medicine that has been on the PBS for some time, its price reduced as a result of past reference pricing and/or other PBS policy decisions and without adjustments for inflation. Unlike other health products and services, the prices of pharmaceuticals are not adjusted to compensate for inflation.

As a new therapy with significant development costs, the challenge to justify a premium over the existing therapy is significant because of the magnitude of the price differential.

It is important to note that this situation will be exacerbated over time because the costs of innovation are increasing. As reference pricing and other cost containment measures drive product prices down, it is increasingly difficult for

[11] Neville, A.M. & Lloyd, J.M. 2004 "Quality of decision-making by the Pharmaceutical Benefits Advisory Committee (PBAC) and the impact on outcomes", Poster presented at ISPOR 2004, Pretium: Sydney.
[12] Ibid.

new products to match these declining prices, let alone demonstrate incremental value.

Medicines Australia submits that this issue alone warrants serious attention.
A potential solution would be to adjust, at least for inflation, the comparator price for the purposes of pharmacoeconomic evaluation.

Systemic inequities arise depending on the therapeutic areas under discussion.
In some therapeutic areas, outcome measures have been subject to greater research and more information is available to present in submissions. Cardiovascular medicine, for example, has benefited from significant worldwide studies that have demonstrated the link between surrogate outcome measures – such as lipid lowering ability or blood pressure control – with long-term mortality and morbidity. In other areas, long-term data is not available. This limits the ability of a new agent to demonstrate the link between the clinical trial outcome measures and longer-term outcomes and creates more uncertainty in the assessment of value.

The nature of some medical conditions presents difficulties because of the 'softness' of the commonly used outcome measures. For example, neurological or mental health conditions rely on physician assessments and some defined measurement tools. While these tools are clinically appropriate, they are notoriously difficult to value in terms of cost effectiveness assessments.

This may also be seen in terms of ability to link 'Quality of Life' measures with demonstrated numerical and incremental cost benefits. For example, within a trial, a product may demonstrate improved quality of life but unless this quality of life measure can be converted to utility scores, this benefit may be largely ignored or at best undervalued in the evaluation process.

3.7 Hierarchy of evidence and surrogate outcomes

The PBAC's strong preference for head-to-head randomised controlled trials establishes a hierarchy of evidence often to the exclusion of additional relevant or supportive data that help inform decision making. This approach also discounts other study designs that might be more appropriate than head-to-head randomised controlled trials for the clinical outcome of interest. An example might be compliance benefits. The practical result is that some benefits are not appropriately valued by the PBAC even though they may be relevant to consumers and to health outcomes and thus the cost to Government.

The PBAC's requirement for high level randomised controlled trials is more stringent than in many other countries and indeed even more stringent than evaluations done by other Australian health authorities such as the National Health and Medical Research Council (NHMRC) which appears to be more flexible in allowing a holistic evaluation of all relevant data and information.

Notwithstanding its reputation for clinical excellence, as a small market (about 1 per cent of the global market for medicines) with a small population, Australia has limited capacity to influence the design of worldwide phase III clinical trials.

Because of regulatory demands for increasingly larger trial sizes, worldwide trials have become common and larger countries' requirements will always dominate over unique Australian requirements. It is therefore unrealistic to expect head-to-head randomised controlled data against a comparator appropriate for Australia for all medicines submitted for PBS listing.

Other global trends include fast completion of phase III trials for regulatory filing and, as a result, surrogate endpoints are often used in clinical trials for some disease areas. While the industry has increased its spend on large clinical studies with long-term final outcomes data, such studies are rarely complete at the time of PBS listing.

To meet the PBAC's requirement for final outcomes, such as survival and quality of life end points, the duration of clinical trials would need to be extended from 18 months or 2 years in most cases to decade/s (for example for HIV and oncology medicines or medicines treating chronic and progressive disabling diseases). This would be prohibitive from cost and ethical perspectives.

Lack of flexibility in the hierarchy of evidence encourages a cost minimisation approach, which in turn drives reference pricing, and lower prices for new products. In other words, if the appropriate data are not available or are not optimal by PBAC standards, options open to applicants are limited. If a difference cannot be demonstrated, an applicant can only agree to a cost minimisation approach. But even these are not straight-forward. It remains rare for global organisations to conduct head-to-head randomised trials, available at the time of registration (or just after) particularly comparing to a product within the same class. This is usually due to the concurrent development timelines of such agents. Thus, at the time of PBS application, head to head assessments, even supporting a cost minimisation basis, may not be available.

For many agents, the only option may be an indirect (or cross study) comparison. These are difficult and carry uncertainty, which again may be a reason for PBAC rejection. While it is willing to consider cross study comparisons, the PBAC has:

- A strong preference for head-to-head randomised controlled clinical trials; and
- Is most influenced by the results of the most rigorous randomised trials.[13]

It is exceedingly rare for the PBAC to accept an argument for differentiation between products using indirect comparisons. Even if the inherent uncertainty can be overcome, this again encourages the cost minimisation approach, again facilitating reference pricing.

Unlike the PBAC, the PBPA will resort to indirect comparisons when conducting annual therapeutic group price reviews. The PBPA will use sample data from GP scripts – an even lower level of clinical and cost effectiveness evidence - as the basis for statistical comparison and price adjustments. This inconsistency points to a lack of coherence in the PBS system.

[13] Sansom, p197

3.8 Uncertainty

Dealing with uncertainty in any decision-making process is a fact of life in business and in government. An example of uncertainty arises in considering the interpretation of statistical tests reported in clinical studies. The PBAC tends to reject any clinical benefits claimed on anything outside the 95 per cent confidence interval or any p value greater than 0.05. Cost effectiveness evaluations built upon such p values are also considered irrelevant and not statistically important. A review of statistical literature reveals that this approach is rather arbitrary in that, for example, a p value of 0.06 still reflects a very significant result.

Uncertainty then becomes a reason for the PBAC to recommend against a listing or impose restrictions on use or suggest a price volume agreement to the PBPA.

Submissions for new medicines made on a cost effective basis have less chance of achieving successful listing. Generally, submissions made on a cost minimisation basis, where a company is not asking for a higher price over the existing comparator are more likely to be successful in achieving listing on the PBS. Cost effectiveness submissions, where a company may request a price increase over the comparator based on some improvement on the comparator are less likely to be successful. For example, one study found that in one period (2000 and 2001) 92 per cent of cost minimisation submissions were successfully listed, compared with 63 per cent of submissions using a cost effectiveness approach[14]. More recent data indicates that only 42 per cent of cost effectiveness submissions for new classes of drugs, new chemical entities and new indications are successful, compared with 88 per cent for cost minimisation submissions[15].

The industry believes that the PBAC should adopt a more flexible and realistic approach to dealing with uncertainty in its decision-making. There is a range of academic papers proposing different methodologies for decision making in the face of uncertainty. These should be evaluated as a basis in the future for the approach by the PBAC.

3.9 Restrictions

In recent times, the PBAC has recommended products for small subsets of the TGA approved indications. This is intended to target those patients for whom cost effectiveness is greatest. Applicants may indeed choose to request restrictive listings in an attempt to gain the prices acceptable to global organisations. The increasing restrictions placed on prescribing of newer agents causes difficulties for prescribers, patients, government and industry alike, with few parties satisfied with the outcome.

Methodologically, such restrictive listings, or the suggestion for such listings, places different data requirements on the local organisations compared with global/parent organisations. Trials are still mainly conducted for regulatory purposes and with the appropriate patient populations consistent with that

[14] Neville, A.M. & Lloyd, J.M. 2004 "Quality of decision-making by the Pharmaceutical Benefits Advisory Committee (PBAC) and the impact on outcomes", Poster presented at ISPOR 2004, Pretium: Sydney.
[15] M-TAG 2004 *Australian Drug Reimbursement and Market Analysis Monitor, August 2004*: Chatswood, p. 4.

regulatory indication. When a subset population is suggested, the company simply may not have the data required in the subgroup required or, if it is available, may be of insufficient power to demonstrate differences. The impact of restrictions is discussed further in Section 4.

3.10 Keeping pace with advanced methodologies and approaches

The industry perceives that the PBS has not kept pace with advanced methodologies and approaches to assessing health technologies and their cost effectiveness.

For example, the PBAC discourages the use of cost-benefit analyses, a technique used increasingly in other countries. The industry is concerned that indirect costs and benefits (eg reduced carers' costs, enhanced productivity) and cost offsets (eg reduced hospital costs) are not given due consideration.

This limits the expression of value that may be attributed to pharmaceuticals. There are numerous studies highlighting the importance of pharmaceuticals in preventing expensive healthcare costs, enabling productivity gains and/or return to active social living (see Sections 8 and 9). Yet the current process does not adequately value these benefits and indeed comments about the *financial* sustainability of the PBS, namely the costs of the PBS, are invariably made in the absence of the contextual framework of benefits.

3.11 Issues for the industry

Satisfying Australia's different methodologies for cost effectiveness analysis and requirements for data is costly for industry, in terms of:

- The costs of conducting specific trials in Australia to gather the data required by the PBAC;
- The subsequent delay in making a submission for PBS listing and thus reaching the market; and
- Highly skilled, specialised staff dedicated to analysing and modelling trial data and preparing submissions.

As a consequence, companies lose the efficiencies associated with conducting worldwide trials, and yet increasingly these are the trials which have the potential to demonstrate differences. It is often said that if a difference cannot be demonstrated, then a price premium is not warranted. While that principle may be appropriate, the barriers in place to demonstrate differences are so significant that they discourage applications for incremental pricing.

Uncertainty has been discussed in the context of PBAC considerations. However, the industry also confronts 'uncertainty' because medicine prices can decline significantly over relatively short periods of time, even while patent protected. For example, the price of Zoloft has significantly eroded in spite of ongoing patent protection. Listed in 1994 at a dispensed price of $89.78, by 2004 its dispensed price has been reduced by more than 60 per cent to $34.84.
Measures that have the most deleterious pricing impacts include:

- The WAMTC methodology, which has a ratcheting effect on prices;
- Use of low priced comparators in the cost effectiveness process;

- The use of off patent comparators in the cost effectiveness process;
- The use of generic price reductions to drive price reductions in linked (on patent) agents after PBS listing;
- The use of 'automatic' price reductions when a new indication is listed; and
- The push for price volume agreements, placing any financial risk on the industry, where prescribers control volumes rather than industry.

These devalue both the innovation and the intellectual property rights attached to the innovation. This is discussed further in the Section 4.

Pricing issues are becoming more problematic. Other countries are increasingly referring to Australia's prices, particularly within the Asia Pacific region. In response, global companies are becoming increasingly reluctant to list products in Australia because of the potential flow-on effect to prices in other countries and are increasing controls on what is considered to be acceptable pricing.

4. THE OUTCOMES OF AUSTRALIA'S HTA ON ACCESS TO MEDICINES AND ON REWARD FOR INNOVATION

"The Commission has found that delays, volume restrictions, complex administration processes and the current application of the main pricing tool, cost effectiveness analysis, are reducing the welfare of consumers by denying them timely access to some drugs and by rationing the use of others…the Commission has found that there is a case for general Government reform to improve the PBS environment" – Industry Commission, 1996.[16]

Nine years ago, the predecessor to the Productivity Commission highlighted the adverse impact of health technology assessment (cost effectiveness analysis) on access to medicines. As described in earlier sections, since then the situation has not improved and, if anything, has deteriorated, in terms of both timeliness to market and accessibility by the Australian community.

4.1 The impact of restrictions on access to medicines

The Australian community's access to the new technology represented in innovative medicines relies on the decisions of global companies to make the medicines available in Australia, given that the vast majority of medicines are developed overseas.

Some medicines are not being made available to the wider community in Australia due to the unacceptably low prices being offered here. Furthermore, the provision of disclosure for PBAC decisions, particularly rejections, has meant that some local subsidiaries are prevented by their global headquarters from making a submission until there is certainty for outcomes in larger markets.

In addition, as the true market in Australia is those products listed on the PBS, the Government's increasing tendency to restrict the conditions for PBS listing means that market access for innovative products is shrinking. As leading global pharmaceutical companies have pricing structures based on effective recognition of the value of biomedical innovations and recovery of the approximately US$800 million investment required on average to develop an innovative global cure, the Australian industry is experiencing serious economic pressures. This imperative has pushed companies into accepting PBS listing for increasingly narrow indications, despite the existence of generally broader TGA indications.

Restricted listings arise in two key ways with the end result being that, in many cases, access to innovative medicines is being limited for patients who could benefit from products that provide therapeutic advantages over older existing therapies, as follows.

The stringent pharmacoeconomic regime employed by the PBAC to list medicines on the PBS places companies in a situation where in order to achieve a price which is acceptable to their overseas head office, they must present evidence to show that the medicine under consideration is more effective in a

[16] Industry Commission 1996 *The Pharmaceutical Industry: Volume 1*, AGPS: Canberra, pp. lv-lvi.

particular subset of the patient population who would benefit from the medicine. During this process, the company may have no choice but to accept a restricted usage to achieve a better price. If it cannot develop an acceptable position, it will not achieve an acceptable price, the medicine may never be reimbursed and therefore may not be introduced into the Australian market. It should be noted that the demands of cost effectiveness are becoming increasingly difficult to meet, with no price adjustments, such as CPI adjustments[17], allowed for comparator products, many of which have been off patent for many years. The result has been a downward price spiral for innovative medicines.

Alternatively, the PBAC, in considering a company's submission, may decide that a medicine is not cost effective or has the potential to 'blow out' the PBS budget unless it has restrictions placed upon its usage. As described earlier, these restrictions can take several forms.

As a result, Australian patients experience restricted access to innovative medicines that could provide broader therapeutic benefits beyond the narrow disease and/or conditions approved by the PBS. This is further exacerbated by the fact that the PBS indication for many medicines is a smaller subset of indications that have been approved by the TGA.

Figure 1

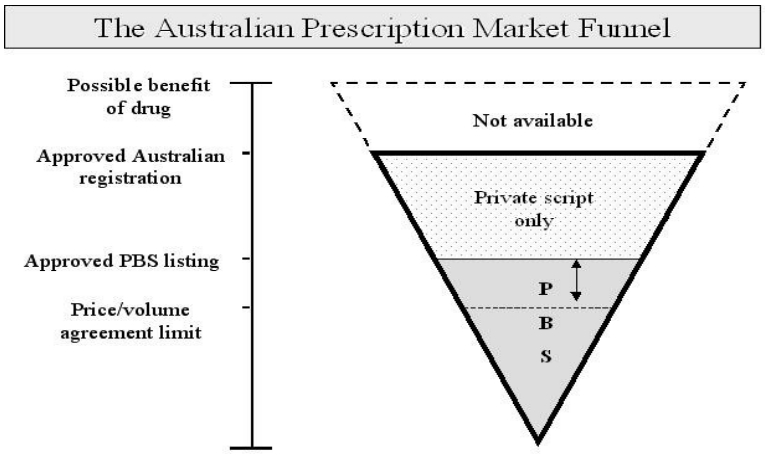


Table 3 below compares the TGA indication and the PBS indication for a sample range of medicines. It is clear from this table that products are often reimbursed for only a subset of the indications approved by the TGA.

[17] CPI may not be the most appropriate index for price adjustment of medicines. This is discussed further in Australian Institute of Health and Welfare (2004) *Health Expenditure 2002-03*, Canberra, p. 67.

Table 3: Percentage of TGA indications approved for PBS reimbursement

Product	Therapeutic area	Estimated proportion of TGA indication reimbursed (%)
Olanzapine	Schizophrenia, bipolar disorder	60-70
Clopidogrel	Prevention of vascular events	20
Ipratropium	Asthma	50
Omeprazole	Ulcers and GORD	66
Paroxetine	Depression, OCD, panic	85
Lansoprazole	Ulcers	25
Valaciclovir	Antiviral	25
Alendronate sodium	Osteoporosis	50
Ranitidine	Ulcers	70
Anastrazole	Hormonal	50
Goserelin	Hormonal	43
Donepezil	Alzheimer's Dementia	50

Critics may argue that these restrictions result from companies electing not to submit for broader indications or submitting data which does not satisfy cost effectiveness for use in the broader indication.

The alternative view is that price erosion sets the hurdle too high for new products with broad indications. As prices of comparator products fall, companies have to produce evidence to justify a larger price premium than would otherwise be the case if the price of the comparator product had remained static. This does not mean that the new product is not very useful and desirable in terms of outcomes but rather the drop in comparator price makes the justification prohibitively difficult. The result, over time, is companies seeking narrower PBS indications based on data in specified populations.

This situation becomes a downward trend that will continue to restrict access by Australian patients to medicines that may well be very appropriate for them in terms of delivering better health outcomes.

Restrictions on access will, in all probability, ultimately prove to be counterproductive in terms of costs and health outcomes. There is a strong body of international evidence about the impact of restrictions on medicine usage. More than 30 international studies have concluded that the primary effect of restrictions is to shift, not reduce, health care costs:

- A US study compared health care spending in 20 states with restrictive formularies with spending in 30 states without such restrictions – it found that while medicine costs declined 13 per cent in states with restrictive formularies, costs for physician services rose 28 per cent and cost of hospital service rose 39 per cent;
- The interrelationship in the elements of the health 'pie' has been vividly shown through the actions of health authorities in the US. In New Hampshire, limits on coverage for the costs of psychotropic medicines resulted in a reduction in the use of these medications, which caused a doubling of the rate of nursing home admissions among chronically ill elderly patients; and
- Similarly, a decision to remove an ulcer-healing medicine, cimetidine, from the Medicaid formulary in the State of Virginia, on grounds of cost,

led to a rise in hospital in-patient treatments for peptic ulcer and actually increased the costs of treating the average patient.

More importantly, decisions on which medicines are eligible for subsidy are often taken without full regard to what is important to individual patients (patient benefits) or to the community as a whole:
"…because individual consumers place different values on the benefits of different medicines, which are not necessarily reflected in the cost effectiveness calculation, they may be willing to pay more for a drug (or particular indication) than the PBS cost effective price. As a consequence, some consumers will be denied access to drug (or particular indications) for which they may be prepared to fund the difference between the PBS cost effective price and the price acceptable to the company. This inaccessibility can result in a loss in public welfare". [18]

Delayed, restricted or no access also affects industry viability. It is often argued that the PBS provides guaranteed volume to pharmaceutical manufacturers which makes up for the low prices received. However, access restrictions and delays impact on volume to the extent that this argument is not correct.

In addition, reference pricing mechanisms in the PBS directly impact on the effective patent life (EPL) of innovative medicines. This situation is unique to Australia due to the dominance of and dependence upon a single government purchaser. In most, if not all major markets including the US and UK, product availability and launch occur immediately following regulatory approval. In Australia, EPL can arguably be defined as the patent term remaining from PBS listing to patent expiry, rather than regulatory approval as is currently the case.

It would appear that the lag between TGA approval and PBS listing is growing as it is now taking longer to achieve PBS listing. Analysis of this data suggests that the time lag between TGA approval and PBS listing is extremely variable. Recent evidence suggests that in 2004, the average time a submission for new classes of drugs, new chemical entities and new indications took from positive ADEC[19] approval to positive PBAC recommendation was 14.8 months, while the average time from positive PBAC recommendation to listing was 8 months[20].

This shortcoming was confirmed by the findings of the post-PBAC review. Delayed decisions affect innovative products disproportionately as they invariably take longer to achieve listing. Not only do delays serve to reduce effective patent life, they may also serve as a de facto cost containment measure.

The impacts of the PBS on volume and effective patent life, when coupled with some of the lowest prices in the OECD for innovative medicines, detract from the intent of the fourth pillar of the National Medicines Policy - a viable industry, and undermine the other three pillars through adverse impacts on access to medicines for the community.

[18] Industry Commission 1996 *The Pharmaceutical Industry, Vol. 1*:, Canberra , p.216.
[19] Australian Drug Evaluation Committee of the Therapeutic Goods Administration.
[20] M-TAG, forthcoming, *Australian Drug Reimbursement and Market Analysis Monitor, December 2004*: Chatswood.

4.2. The impact on reward for innovation

Achieving reasonable prices on market entry provides return on companies' investment in innovation, which in turn will encourage further investment in innovation including R&D in Australia.

However, as the Productivity Commission made clear in its 2001 report on international pricing, prices paid for branded and innovative medicines in Australia are among the lowest in OECD countries. This is the outcome of cost containment policies. Most recently, the US Department of Commerce found that Australia's prices for patented medicines are amongst the lowest in the OECD, equivalent to those paid in Poland[21].

Increasingly stringent cost controls have reduced returns on investment and/or reduced patient access to advanced medicines. These methods:

- Reduce the price and/or usage of an innovative medicine when it is first listed on the PBS; and
- Erode the price of PBS medicines throughout their patent life. In some cases, the price reductions sought jeopardise the continued PBS listing of the medicine (and hence patients' access to the medicine).

In most developed country markets, a strong intellectual property regime provides a level of exclusivity for patented products that supports further investment in innovative medicine discovery. In Australia this return is significantly curtailed firstly, by the low price received at listing; and secondly, by the time more comprehensive data has been amassed to differentiate the product to support a higher PBS price, the product will have reached or be close to, patent expiry – thus exposing it to generic competition.

Yet, in Australia, if the company cannot demonstrate from limited data at the time of listing, through evidence from its clinical trials, that it is better than the comparator medicine, it will only be able to obtain the same price as the older comparator. If the company can demonstrate that its medicine is better than the comparator medicine, it may get a small premium. The comparator price is also not inflation adjusted, making it increasingly difficult for companies to prove cost effectiveness for new products.

The value of innovative medicines in the PBS is suppressed by:

- The use of low-priced comparators in the cost effectiveness process;
- The lack of inflation adjustment for comparator medicines in the cost effectiveness process;
- The use of off-patent comparators in the cost effectiveness process; and
- Restricting the usage of a medicine.

Through reference pricing, generic price reductions are used to drive price reductions in linked (in-patent) medicines, post PBS listing. While innovative medicines, which are still protected by patents, are treated as 'the same' as a

[21] Department of Commerce 2004 *Pharmaceutical Price Controls in OECD Countries: Implications for US Consumers, Research and Development, and Innovation*, International Trade Administration: Washington, p. 15.

much older, off-patent comparator, these medicines are not bioequivalent and therefore not generally interchangeable. From a cost-containment perspective, reference pricing during the active patent life accelerates price decreases that would only occur once a product's patent expires.

However, this has the adverse effect of seriously devaluing innovative medical discoveries which can offer important benefits to Australian patients in terms of efficacy in curing or treating a life-threatening or disabling disease; improved safety and/or reduced side effects; and lifestyle (e.g. faster recovery times, resumption of normal activities, etc.)

The link between patented and off-patent medicines:

The link between patented and off-patent medicines can occur at the time of initial PBS listing because the "comparator" used in the cost-effectiveness process is an off patent medicine; and throughout the life of the patented product, because of Australia's unique reference pricing system:

"The use of reference pricing in Australia is more likely to lead to greater price suppression in the local market than a number of other OECD countries due to two design features:

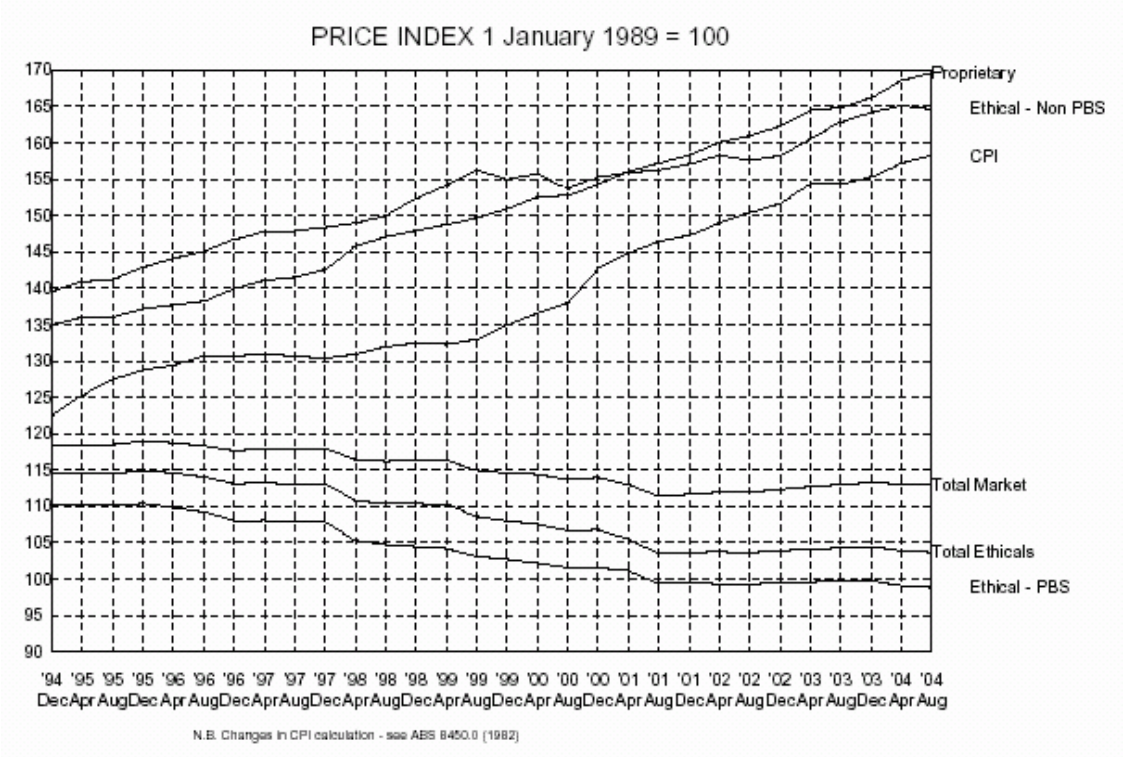
- unlike many other countries, Australia's adaptation of reference pricing includes patent as well as off-patent pharmaceuticals in the groups. Off patent pharmaceuticals…are likely…to constrain patent drug prices;
- whereas Australia uses the minimum price as the benchmark, some other countries use the average group price" *(Productivity Commission draft research report "Evaluation of the Pharmaceutical Industry Investment Program", Dec 2002, p.3.7)*

The report by the US Department of Commerce found that low prices paid for innovative, patented medicines in a number of OECD countries adversely impacted on global innovation in medicines. It found that if these countries paid equivalent US prices for patented medicines, global pharmaceutical R&D would increase by up to $10 billion per annum, and could lead to the development of three to four additional new medicines each year[22].

The impact of government monopsony purchasing power and policies on prices for medicines are vividly reflected in Figure 2, based on the Pollard Index. The index for prescription medicines covered by the PBS (Ethicals-PBS) shows that not only have prices not kept pace with the CPI increases, but also the gap between the two indices has been widening, demonstrating further price deterioration for PBS-listed prescription medicines. By contrast, prices for non-PBS Ethicals and proprietary medicines, where there has been no government intervention, have maintained parity with CPI growth.

[22] Department of Commerce 2004 *Pharmaceutical Price Controls in OECD Countries: Implications for US Consumers, Research and Development, and Innovation*, International Trade Administration: Washington.

Figure 2: Pollard Index of Pharmaceutical Prices Dec 1994 – August 2004



Source: IMS Health.

The effects of the cost containment policy have also been manifested in many other ways. Firstly, some new medicines are receiving the same or a much lesser price, in real terms, than medicines listed 10 to 20 years ago, as shown below.

Figure 3

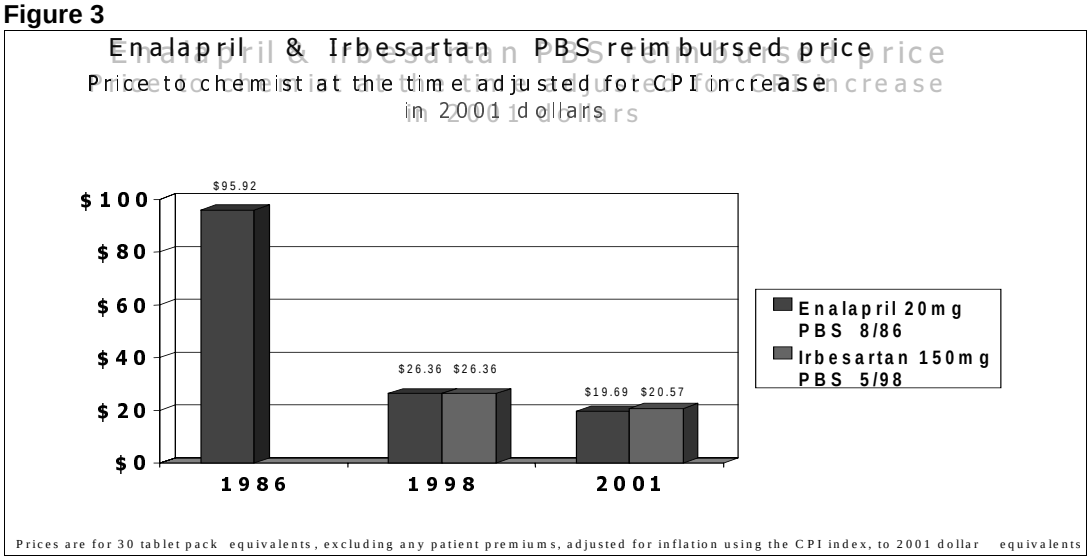


Figure 3 compares ACEI, enalapril with A2A, irbesartan. The A2As are a new class of medicines for the treatment of high blood pressure. To achieve the PBS listing, the comparator product was enalapril, a product launched 16 years ago. Launched in 1998, the real price of irbesartan was less than the price of enalapril at its launch in 1986.

Secondly, compared with other countries in the world, Australia's new patented therapies decline in price, whereas prices in other countries either stay reasonably steady or increase. This is due to Australia's practice of referencing prices of some patented medicines to the price of generic medicines. In other countries, prices decline significantly only on expiry of the patents. *"Using the power that governments have to regulate drug prices, or pharmaceutical expenditure, presents something of a dilemma. If prices for patented and branded products are set too low, the incentives for further innovation will be diminished. This dilemma is complicated by the fact that the market for important pharmaceuticals is a global market. Successful innovation has some of the characteristics of a public good. Therefore, the costs of R&D also need to be shared at the international level"*[23].

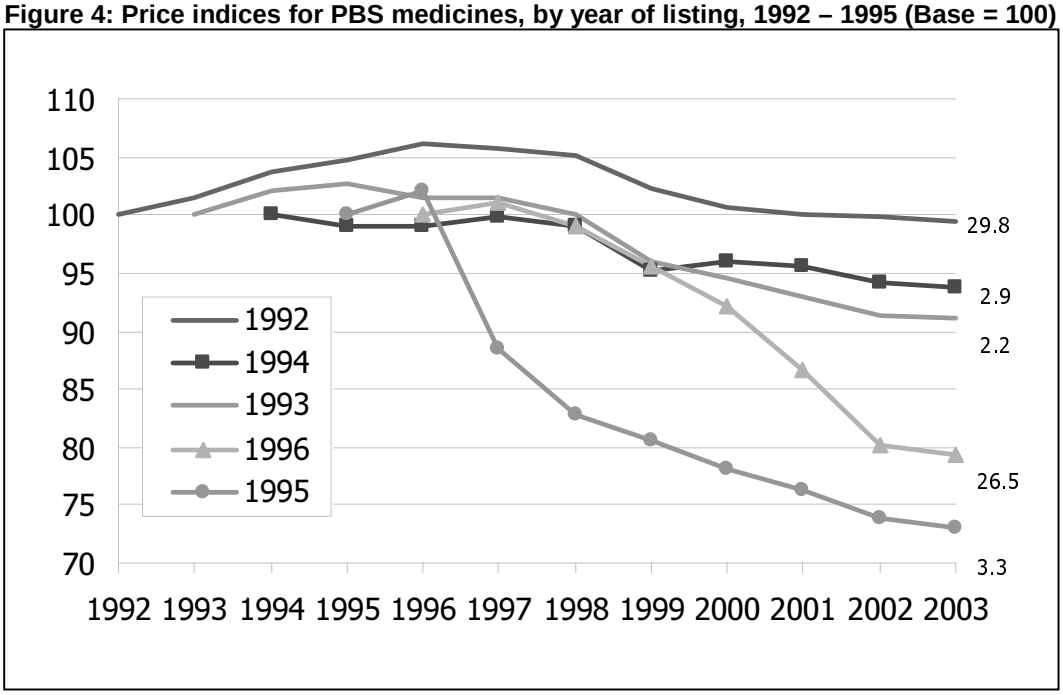
Analysis by the CSES has shown that there is a growing lack of coherence in the PBS, with:

- Inconsistency between cost effectiveness analysis and reference pricing; and
- Imbalance between on-patent and off-patent pricing.

Its analysis (see Figures 4 and 5 on the following page) has found that headroom for innovative medicines is being created by price erosion in other innovative medicines; that prices for innovative medicines are low by world standards, and have been falling, with falling prices concentrated in the medicines listed in more recent years.

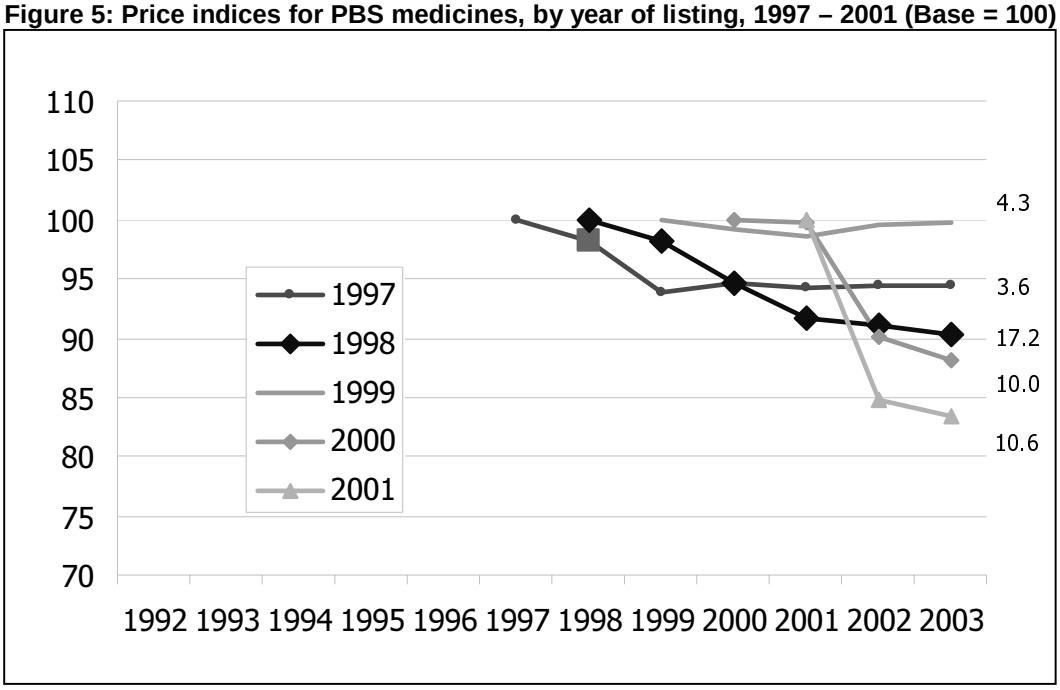
[23] Jacobzone, S. 2000 *Pharmaceutical Policies in OECD Countries: Reconciling Social and Industrial Goals*, Labour Market and Social Policy Occasional Papers, No. 40, OECD: Paris, p. 5.

Figure 4: Price indices for PBS medicines, by year of listing, 1992 – 1995 (Base = 100)



Source: Centre for Strategic Economic Studies

Figure 5: Price indices for PBS medicines, by year of listing, 1997 – 2001 (Base = 100)



Source: Centre for Strategic Economic Studies

5. PBS EXPENDITURE OVER THE NEXT FIVE YEARS – TRENDS AND FUTURE SUSTAINABILITY

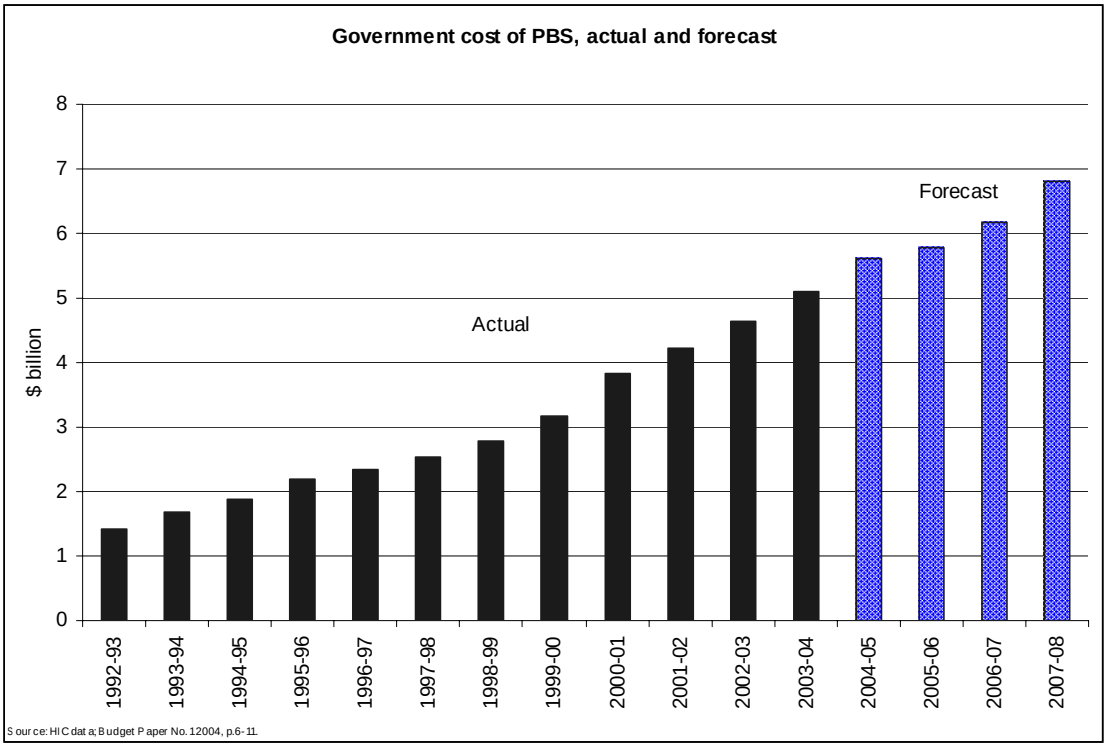
The PBS is one of the major areas of health expenditure in Australia. Questions are often raised about the future of the PBS and its long-term sustainability. In looking at the future of the PBS, it is important to consider a number of issues likely to impact on future PBS spending. These include the estimates of what PBS expenditure is likely to be in the future, the role of the PBS in Australia's health system compared to other countries, the impact of patent expiry on older medicines, the impact of new medicines becoming available and technological developments.

Several policy options that may help ensure the sustainability of the PBS into the future, both in changes to PBS co-payments and broader medicine funding options, are also canvassed here. All of these factors are important to a discussion of the future of the PBS over the next few years and in the longer-term.

5.1 Future growth in the PBS

Expenditure on the PBS is likely to grow over the next five years. This is due to a range of factors including an ageing population, the identification of new treatments, latest technology medicines developed to treat a range of health conditions and the demand from consumers for access to the latest treatments. Estimates of future PBS spending vary.

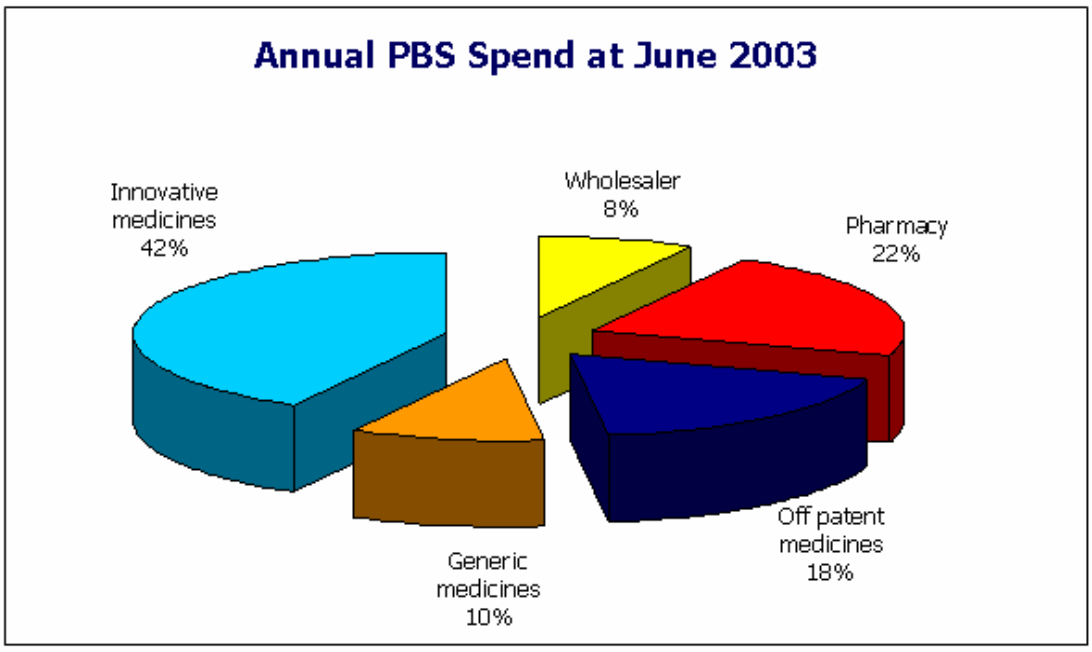
Figure 6



The 2004-05 Federal Budget papers suggest growth in the PBS will continue until 2007-08 (Figure 6). Since 1992-93, growth in Government spending on the PBS has averaged 12.5 per cent per annum, not adjusted for inflation. From 2004-05 until 2007-08, the Government's forward estimates forecast a growth rate for the PBS of 7.5 per cent per annum. In particular, in 2005-06, Government spending on the PBS is forecast to grow at 3.4 per cent, probably in part due to an expected fall in usage related to the introduction of higher co-payments from 1 January 2005. (This is discussed in more detail in Section 6).

The PBS funds a range of activities devoted to delivering medicines to the end-user: the patient. It is not always recognised that the cost of the PBS is not only the cost of the medicines themselves, but the services provided by wholesalers and pharmacists in delivering the medicines manufactured by pharmaceutical companies to patients. Around 70 per cent, or less than three quarters of the PBS is actually spent on medicines themselves (Figure 7). Innovative medicines account for 42 per cent of PBS spending, off-patent medicines 18 per cent and generic medicines 10 per cent. This means that patented medicines account for less than half of the cost of the PBS. The remaining 30 per cent of the PBS is spent on distribution through wholesalers' margins, pharmacists' fees and other marketing costs.

Figure 7



Source: Medicines Australia.

While the price of medicines themselves are obviously one driver of the growth in PBS spending, so too are these other components of the PBS. For example, the payments made to pharmacists out of the PBS are indexed to the CPI, protecting the real value of these against inflation. This is in contrast to the price of prescription medicines on the PBS which have failed to keep pace with inflation due to the system of reference pricing and cost effectiveness evaluation.

The result is that aggregate prices for PBS medicines have shown little increase and have fallen since the mid-1990s. *"The tendency for price falls to be concentrated in the new medicines is a particularly notable feature of the Australian system"*[24]. See Section 4 for a more detailed discussion of pharmaceutical prices.

In terms of future influences in PBS growth, a number of components are likely to contribute to the growth in the PBS. Medicines themselves will be a contributor to the PBS, although the prices of these are not keeping pace with inflation, while other components in the supply chain that deliver the medicines to the consumer will also be a contributor, particularly as they are indexed to grow with inflation.

Much of the previous policy reform of the PBS has focused on the beginning of the distribution chain. There may be scope for reducing transaction costs and achieving efficiency gains through a review of the operation of the whole value chain. The industry would be prepared to participate in such a review.

While concerns are sometimes raised about the growth in the PBS, international comparisons reveal that Australia's level of spending on pharmaceuticals is not unusually high. In 2001, Australia spent 1.3 per cent of GDP on pharmaceuticals, around the mid- to lower-range of spending compared to other OECD countries (Table 4). Moreover, its level of overall spending on health (9.1 per cent) is higher than many other OECD countries.

The result is that the ratio of Australia's pharmaceutical spending to its total health spending is not especially high and, if anything, is towards the lower end of the scale.

This suggests that Australia has elected to spend less of its resources on innovative medicines and more on other health treatments compared to many other OECD countries.

[24] Sweeney, K. 2004, *Review of Findings: Australian Pharmaceutical Pricing in a Global Context*, Working Paper No. 19, Pharmaceutical Industry Project, Centre for Strategic Economic Studies, Victoria University of Technology: Melbourne, p. 2.

Table 4: OECD Countries' spending on pharmaceuticals & health as a share of GDP 2001

Country	Spending as a share of GDP (%) on …		Ratio pharmaceuticals to health spending
	Pharmaceuticals	Health	
Slovak Republic	1.9	5.6	0.34
Hungary	2.1	7.4	0.28
Italy	1.9	8.3	0.23
Korea	1.3	5.9	0.22
Czech Republic	1.6	7.3	0.22
Spain	1.6	7.5	0.21
France	2.0	9.4	0.21
Mexico	1.2	6.0	0.20
Japan	1.5	7.8	0.19
Canada	1.5	9.4	0.16
Greece	1.5	9.4	0.16
Austria	1.2	7.6	0.16
Finland	1.1	7.0	0.16
Australia	**1.3**	**9.1**	**0.14**
Iceland	1.3	9.2	0.14
Germany	1.5	10.8	0.14
Sweden	1.2	8.8	0.14
United States	1.7	13.9	0.12
Luxembourg	0.7	5.9	0.12
Switzerland	1.2	10.9	0.11
Netherlands	0.9	8.5	0.11
Ireland	0.7	6.9	0.10
Denmark	0.8	8.6	0.09

Source: OECD Health Data 2004.

Projected lower growth rates in PBS spending through the second half of the 2000s do not take into account patent expiries set to happen in the next few years. With patent expiry, several major therapeutic areas in the PBS, such as statins for cholesterol lowering and selective serotonin reuptake inhibitor inhibitors for mental illness, will see the entry of generic versions of patented medicines, with likely consequent price reductions. The Government's forward estimates for the PBS do not take this impact into account. In evidence to Parliamentary Senate Estimates hearings, DoHA officials made clear that the estimates do not account for medicines going off patent[25]. Nor do the forward estimates take account of new molecules coming on stream in individual therapeutic areas. The PBS is an important area of public expenditure and should continue to grow with Australia's development and community expectations about access to the latest innovative medicines. However, it could be that the actual spending levels could be quite different from the Government's forecasts.

[25] Senate Community Affairs Legislation Committee 2004 *Hansard: Budget Estimates*, 2 June, http://www.aph.gov.au/hansard, p. 110.

5.2 Problems in forecasting future growth

DoHA has indicated to the industry in the past that it is difficult to forecast future growth in the PBS. Medicines Australia would welcome the opportunity to work more closely with DoHA on estimating future PBS expenditure by providing information on medicines in the product 'pipeline'. Medicines Australia has in the past recommended that the Government and industry could work jointly in better estimating future PBS growth.

Government has in the past occasionally seen multiple medications become available for patients in a short period of time. This has in some cases resulted in unexpected movements in the total cost of the PBS and the accompanying concerns about the capacity to accurately forecast expenditure in this area.

Through dialogue, government and industry could identify what information would be of use to government in forecasting accurately and how that information would best be collected. By delivering transparency and certainty to government, greater confidence in the partnership between industry and government can be built.

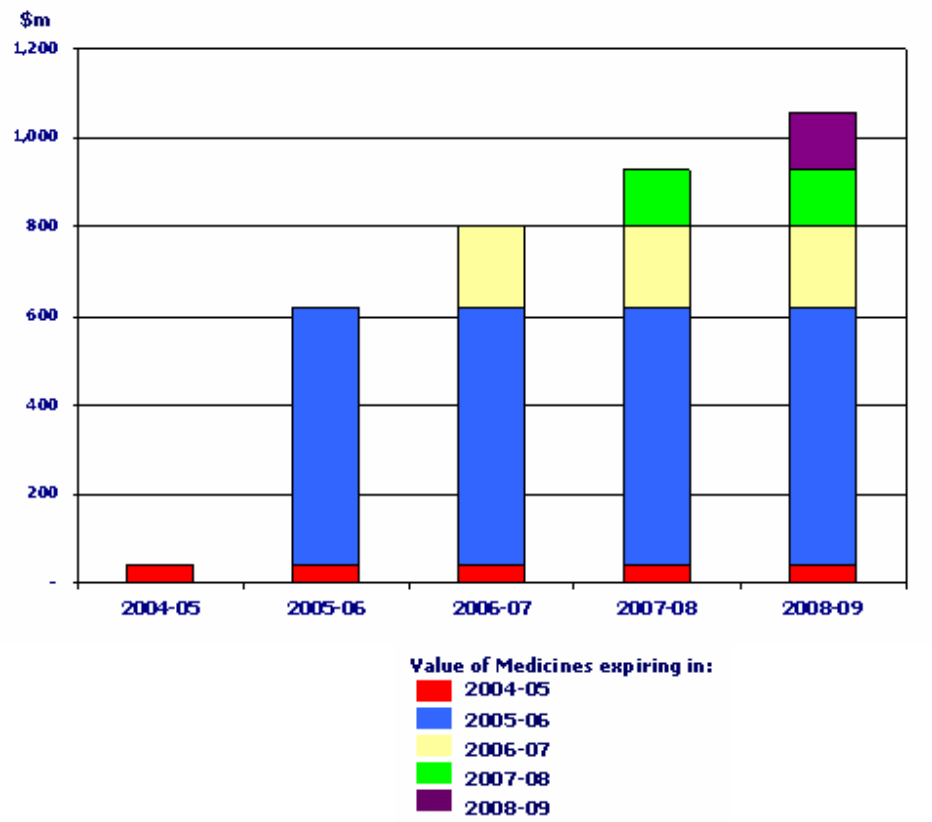
Medicines Australia and the National Centre for Social and Economic Modelling (NATSEM) have developed the MediSim model of the PBS through an ARC Linkage grant. MediSim is a micro-simulation socio-economic model of the Australian pharmaceutical market designed to model the PBS and assess the distributional impacts on households of changes in the PBS due to changes in the market or public policy.

The MediSim model includes 5 year forecasts of the PBS, which try to take account of the impact of older molecules going off patent, as well as new molecules becoming available on the PBS. Medicines Australia and NATSEM are currently looking to update and verify the validity of these forecasts.

5.3 Potential impact of approaching patent expiries

The Centre for Strategic Economic Studies (CSES) has also conducted some research on the future of PBS spending. These studies take into account the impact of medicines going off patent. The CSES estimates, that through the period 2004-05 through to 2008-09, the cumulative value of PBS medicines coming off patent will be just over $1billion, including around $600 million in 2005-06 when statins start coming off patent (Figure 8).

Figure 8: Cumulative value of medicines coming off patent over the next five years (2001-02 prices).



Source: Centre for Strategic Economic Studies.

Not taking these savings into account, nor anticipating new, patented medicines likely to be listed on the PBS, means that the Government's forecasts are less likely to capture all the influences on PBS expenditure in future years.

5.4 Potential impact of changes in generic pricing and utilisation

Australia has relatively low uptake of generic medicines compared with other countries. *"The highest market share for generics is found in countries where the Industry historically had the greatest pricing freedom, including Germany, the Netherlands, the UK and the US. Where systems of price control have been in place, such as Australia, generics have a smaller market share"*[26]. For example, in 2001, generics accounted for 45 per cent of all prescriptions filled in the US market[27]. However, they only accounted for 8.4 per cent of prescriptions filled in the US. Brand name medicines accounted for 55 per cent of all scripts and 91.6 per cent of consumer spending. In the UK 70 per cent of scripts are written generically[28]. The shares in the Netherlands and Germany are said to be around 30 per cent and 50 per cent respectively[29]. In Australia by contrast, only 10 per cent of the PBS is met by generics (see Figure 8 on previous page). Similar

[26] Lofgren, H. 2002 *Generic Drugs: International Trends and Policy Developments in Australia*, Working Paper No. 10, Centre for Strategic Economic Studies, Victoria University of Technology: Melbourne, p. 3.
[27] Ibid, p. 3.
[28] Ibid, p. 4.
[29] Ibid, p. 4.

trends were identified were identified by the US Department of Commerce, which found that Australia had a relatively low utilisation of generics compared with other countries like Canada, Germany, Poland, UK and the US[30].

The major reason for this is that in Australia the prices of patent-protected medicines are low. The Productivity Commission undertook a major comparison of Australia's pharmaceutical prices with overseas prices in 2001[31]. It found that prices paid for branded and innovative medicines in Australia are amongst the lowest in the OECD. The US Department of Commerce confirmed this finding. It found that Australia's prices for patented medicines are amongst the lowest of the OECD countries examined[32]. The situation with the prices for generic products is mixed. The US Department of Commerce found that Australia's generic prices are in the mid-to-low range, on a par with the United States and a few other countries[33]. Sweeney found that although Australia's generic prices are low, they are not as low as some other countries[34]. While overall generic prices are in the mid- to low- range in Australia, anecdotal evidence suggests that the price of a number of high volume generics in Australia may be quite high. The key point is that in Australia generics tend to be relatively more expensive compared to patented medicines because the price of patented medicines is so low.

"While prices of patent-protected drugs are low in Australia compared to other countries ..., generic prices tend to be relatively high. This is because the PBS sets the price of originator brands close to the price of generic brands, thus discouraging the entry of generic suppliers. This is exacerbated by the high level of oligopoly in the generic drugs supply industry"[35].

As an illustration, the brand premium applying to a medicine is the difference between the price charged by an original brand name product and the price of the generic product once a generic version has become available after patent expiry. A quick look through the Department of Health and Ageing's Schedule of Pharmaceutical Benefits (the 'yellow book') will reveal that, in most cases in Australia, the brand premium applying to a brand-name medicine is only a few dollars above the generic price – only a small mark-up. This helps to explain why the uptake of generics is so low in Australia: with innovative, patented prices in Australia being so low, there is little incentive to use generics.

A major reason why the prices for innovative medicines are so low in Australia is because they are linked to the prices of generic products through various reference mechanisms described in Section 2. These mechanisms make it

[30] Department of Commerce 2004 Pharmaceutical Price Controls in OECD Countries: Implications for US Consumers, Research and Development, and Innovation, International Trade Administration: Washington, pp. 23.
[31] Productivity Commission 2001*International Pharmaceutical Price Differences*, Research Report, July: Canberra.
[32] Department of Commerce 2004 *Pharmaceutical Price Controls in OECD Countries: Implications for US Consumers, Research and Development, and Innovation*, International Trade Administration: Washington, p. 15.
[33] Ibid, p. 22.
[34] Sweeney, K. 2004 "Review of Findings: Australian Pharmaceutical Pricing in a Global Context", Working Paper No. 19, Pharmaceutical Industry Project, Centre for Strategic Economic Studies, Victoria University of Technology: Melbourne, p. 3.
[35] Ibid, p. 6.

difficult for companies to obtain reimbursement for newer, innovative medicines in Australia.

Such a pricing policy, where the prices paid for innovative medicines are relatively low compared with prices of generic medicines, would seem at odds with the goal of ensuring Australians' access to the latest medicines available. Compared to other countries, Australia's pricing system for medicines provides relatively greater rewards to older, out-of-patent medicines than it does to the latest patented, innovative medicines.

5.5 The role of new medicines in transforming healthcare

Throughout history, the development of new medicines has transformed health care, saved lives and improved peoples' quality of life. Life expectancy across the world has increased dramatically over the last half a century and this has been due, in no small part, to technological developments leading to the development of new medicines. For example, diseases such as small pox, polio, tuberculosis and measles that once killed many and cost society millions to treat are now eradicated or being controlled by new vaccines developed by the pharmaceutical industry.

Twenty years ago the life expectancy for a patient with HIV/AIDS was not long and quality of life was severely hampered. Through various anti-viral treatments developed by the pharmaceutical industry, today a person diagnosed with HIV/AIDS can expect a much longer life expectancy and higher quality of life than if that person had been diagnosed 20 years ago. Currently there are 82 AIDS medicines available, with an additional 79 currently undergoing clinical trials[36].

Stories such as this highlight the major contribution that medicines make to life. As discussed elsewhere in this submission, medicines make a major contribution to health, economic and social outcomes, as well as offset costs in other parts of the health system.

5.6 The environment for new medicine development

Developing new medicines does not come cheaply and it is becoming more complex and difficult. While the community is looking to the industry to develop cures for illnesses such as Alzheimer's Disease, cancer and HIV/AIDS, the cost of developing new medicines has increased. Estimates of the cost of developing a new medicine vary. The Tufts Centre for the Study of Drug Development estimates that the cost of developing a new prescription medicine is US$802 million[37], or just over $1billion. Other estimates have put the cost of developing a new medicine as high as US$1.7 billion[38] ($2.2 billion).

[36] PhRMA 2004 "Researchers are Testing 79 Medicines and Vaccines for HIV and Opportunistic Infections" *Medicines in Development for HIV/AIDS 2004*, http://www.phrma.org/newmedicines/resources/2004-11-30.146.pdf (accessed 2/12/04), p. 1.

[37] Tufts Centre for the Study of Drug Development 2001 "Tufts Center for the Study of Drug Development Pegs Cost of a New Prescription Medicine at $802 million", *News Release*, 30/11/2001, http://csdd.tufts.edu (accessed 19/2/2004).

[38] Gilbert, J., Henske, P. & Singh, A. 2003 "Rebuilding Big Pharma's Business Model" *In Vivo: the Business and Medicine Report*, 21(10), November, p. 4.

Contributing to the increase in costs is the decline in productivity in research. For example, in 2003 the US FDA approved 21 new medicines compared with 53 in 1996 and the trend is continuing[39].

Developing new medicines is also becoming more risky. The probability of achieving a 12 per cent rate of return on a new medicine has halved from 30 per cent during the period 1995-2000 to 15 per cent in 2000-02[40]. After releasing an average of 59 new medicines per annum between 1998 and 2002, forecasts are suggesting that pharmaceutical companies will release a lower average of 50 medicines per annum from 2003 to 2006[41].

The industry as a whole has to come to grips with the problem that the flow of new medicines is not as strong as in the past. The 'pipeline' of new compounds is not as productive and companies have to assess their business model to deal with the downturn in new product development.

On the other hand, companies are identifying new sources of potential compounds in their search for cures. Gene technologies and bioinformatics are increasingly allowing companies to conduct computer modelling of molecules and their effect on diseases before commencing clinical trials. However there is some debate about the extent to which these new techniques will offset the competitive challenges facing the industry.

The rise of 'biologicals' is also presenting challenges as well as opportunities. Biologicals differ from the more traditional class of medicines because their development is more complex. Basic development is more complex than traditional molecular chemistry; the scale up from bench to production of clinical trial materials may be more complex, and the ultimate scale up to commercial production even more complex. They offer potentially enormous medical benefits for the community in that they are better targeted to particular illnesses (or subsets of patients) and therefore have the potential to be more effective. Biologicals also open up the possibility of a whole new range of treatments for conditions such as arthritis.

However, these types of medicines are expensive. Not only are they are expensive to develop, they are usually targeted at small patient groups, tailored to suit particular diseases or more severe forms of diseases. Novel issues associated with their basic development, scale up, toxicity and manufacture mean it may be more involved to obtain regulatory and pricing approval for these medicines, and they will pose real challenges to reimbursement systems.

Government and industry have the potential to work together to develop improved understanding of these new technologies and how to assess them appropriately.

[39] "Fixing the drugs pipeline", *The Economist*, 11/3/2004, www.economist.com (accessed 18/3/04).
[40] Gilbert, J., Henske, P. & Singh, A. 2003 "Rebuilding Big Pharma's Business Model" *In Vivo: the Business and Medicine Report*, 21(10), November, p. 2.
[41] Ellis, S. 2004 "Drug firms chase healthy little injections" *Australian Financial Review*, 22/4/2004, p. 29.

5.7 New medicines likely to appear on the market

In this environment developing new medicines is becoming more challenging. However, a range of new medicines are currently undergoing clinical trials, meaning that at least some of these are likely to come on to the market over the next five to ten years. Generally, only one in five medicines that begin clinical trials make it to market[42], although some research suggests that the success rate in more recent years could be as low as one in nine[43].

An appreciation of the scope of developments likely over the next five to 10 years can be obtained by an analysis of the website developed by the Pharmaceutical Research and Manufacturers of America (PhRMA), www.innovation.org. This website has details on the individual medicines currently being developed for particular health conditions.

Although the site is based in the United States, it does include products where the development is based in other countries and the information still provides a useful insight into the range of medicinal treatments currently in the pipeline. In this data there may be some small double counting as the same medicine may be being developed for more than one condition. However, the information still provides an indication of where much of the activity in future medicine development is occurring.

For the purpose of this submission, new medicines relevant to the Australian Government's National Health Priority Areas (NHPAs) have been summarised. Although hundreds of conditions are listed in detail on the www.innovation.org website, only those relevant to the NHPAs are examined here. All of the potential new treatments are in clinical trials in humans at the time of writing. This means that by and large all of the earlier stages of research have been completed. The overall results of this analysis are presented in Table 5.

Table 5: New medicines currently in development, by Australia's National Health Priority Area, by stage of development, November 2004.

Condition	Phase					
	I	II	III	Apply*	Other	Total
Asthma	8	20	4	5	1	38
Cancer	56	122	62	4	1	245
Cardiovascular	18	35	20	0	2	75
Mental health	9	16	12	6	3	46
Diabetes	18	20	9	6	3	56
Injury prevention	1		3			4
Arthritis	24	27	17	9	4	81
Dementia	13	6	9	6	0	34
Total	*147*	*246*	*136*	*36*	*14*	*579*

Source: Derived from www.innovation.org (PhRMA website). Accessed 24/11/2004. * FDA application.

[42] PhRMA 2004 *Why Do Medicines Cost So Much and Other Questions About Your Medicines*, http://www.phrma.org/publications/publications/brochure/questions/questions.pdf (accessed 2/12/04), p. 2.
[43] Gilbert, J., Henske, P. & Singh, A. 2003 "Rebuilding Big Pharma's Business Model" *In Vivo: the Business and Medicine Report*, 21(10), November, p. 4.

Cancer clearly has the highest priority in terms of new medicine development. Table 5 shows that of an estimated 579 new therapies being developed for different health conditions, 245 are for one form or other of cancer. Around half of these, 122, are currently at Phase 2 stage of clinical trials, while 56 and 62 treatments are in Phases I and III respectively. Arthritis is the next highest with 81 new treatments currently in the pipeline, closely followed by cardiovascular health with 75 new medicines in the pipeline.

Based on the number of molecules in Phase I, an estimate of the likely number of new medicines making it on to the market can be calculated, using the figure that one in five, or 20 per cent, of medicines that enter clinical trials make it to the market. For example, it could be expected that of the 56 cancer treatments currently at Phase I of clinical trials, around 11 will make it to market over the next five to 10 years. However, with 122 cancer treatments already in Phase II, one would expect that a larger number of these will enter the market sooner. For example, based on Gilbert, Henske and Singh's view that one in five medicines at Phase II will make it to market[44], one could speculate that of the 122 cancer medicines currently at Phase II of clinical trials, 24 could be expected to be on the market over the same time period.

Table 5 also provides some insight into the relative stages of development of different therapies. For example, while treatments for arthritis are reasonably evenly spread across the three phases of clinical trials, in cancer treatments there are twice as many molecules at Phase II than at Phase I or III, suggesting, that there could be a wave of new cancer treatments five to 10 years away.

More detailed results on medicines in the pipelines related to Australia's NHPAs are contained in Table 6. On an individual health condition basis, lung cancer has the most prospective treatments with 52 medicines in the pipeline. The next highest are Type 2 diabetes (48), breast cancer (45), rheumatoid arthritis (42) and prostate cancer (41).

Table 6: New medicines currently in development, by Australia's National Health Priority Area, by stage of development, November 2004.

Condition	Phase					
	I	II	III	Apply*	Other	Total
Asthma	8	20	4	5	1	38
Total asthma	*8*	*20*	*4*	*5*	*1*	*38*
Cancer, breast	12	23	9		1	45
Cancer, cervical	6	5	2			13
Cancer, cervical - prevention		1	1			2
Cancer, Colon	6	3	2	1		12
Cancer, colorectal	7	19	10			36
Cancer, Colorectal Adjuvant Therapy			2			2
Cancer, Colorectal Metastatic		1				1
Cancer, lung	11	28	12	1		52
Cancer, prostate	7	25	8	1		41

[44] Gilbert, J., Henske, P. & Singh, A. 2003 "Rebuilding Big Pharma's Business Model" *In Vivo: the Business and Medicine Report*, 21(10), November, p. 4.

Condition	Phase					
	I	**II**	**III**	**Apply***	**Other**	**Total**
Cancer, Prostate, Early-Stage		2				2
Cancer, skin	3	6	9	1		19
Cancer, Skin, Non-Melanoma			1			1
Cancer, Lymphoma, Non-Hodgkin's	4	9	6			19
Total cancer	*56*	*122*	*62*	*4*	*1*	*245*
Cardiovascular disease	4	1	1			6
Heart disease, other			1			1
Heart failure, acute			1			1
Heart failure, chronic		1	2			3
Heart failure, diastolic		1				1
Stroke	6	8	3			17
Stroke, Acute Ischemic	1	5	2			8
Stroke, Hemorrhagic		2				2
Stroke, Ischemic	1				2	3
Stroke, Prevention in Atrial Fibrillation	2	1	1			4
Peripheral Vascular Disease	1	4	2			7
Peripheral Arterial Disease		5	1			6
Arteriosclerosis	1		1			2
Cardiac disease	1					1
Congestive Heart Failure	1	5	5			11
Congestive Heart Failure (Outpatient Study)		1				1
Congestive Heart Failure, Acute and Chronic		1				1
Total cardiovascular	*18*	*35*	*20*	*0*	*2*	*75*
Depression	7	10	2	3	1	23
Depression, Major Depressive Disorder		2	1			3
Anxiety	1					1
Anxiety, acute	1					1
Anxiety, Generalized Disorder		2	2			4
Bipolar depression			1	1	1	3
Bipolar disorder					1	1
Bipolar maintenance				1		1
Schizophrenia		2	6	1		9
Total mental health	*9*	*16*	*12*	*6*	*3*	*46*
Diabetes - type 1		1		1		2
Diabetes - type 2	18	16	8	3	3	48
Diabetes - type 1 & 2		3	1	2		6
Total diabetes	*18*	*20*	*9*	*6*	*3*	*56*
Injury prevention	1		3			4
Total injury prevention	*1*		*3*			*4*
Arthritis		1	1			2
Arthritis, psoriatic		1	1			2
Arthritis, rheumatoid	12	19	7	2	2	42
Osteoarthritis	2	3	1	3		9
Osteoporosis	9	2	7	4	2	24

Condition	Phase					
	I	**II**	**III**	**Apply***	**Other**	**Total**
Osteoporosis, post menopausal	1	1				2
Total arthritis	*24*	*27*	*17*	*9*	*4*	*81*
Dementia			1	1		2
Dementia, Vascular	2		2			4
Alzheimer's Disease	11	6	6	5		28
Total dementia	*13*	*6*	*9*	*6*	*0*	*34*
Total for NHPAs	***147***	***246***	***136***	***36***	***14***	***579***

Source: Derived from www.innovation.org (PhRMA website). Accessed 24/11/2004. * FDA application.

A proportion of the new medicines being developed will provide patients, governments and the health system new tools to treat some of the most serious health conditions in our community today. In some cases, these will be medicinal treatments that treat illnesses which previously needed to be managed by other parts of the health system. For example, an effective medicine for prostate cancer could remove the need for patients to have radiation therapy or prostatectomy surgery. Or if one of the 28 treatments currently being developed for Alzheimer's Disease proves effective, as well as treating a major illness of an ageing population, it could save much money in other parts of the health system such as aged care costs.

5.8 Potential for further changes in patient co-payments

With all of these changes taking place, the sustainability of the PBS is a key issue for the future. There is a range of options available to Government to reform PBS funding arrangements through changes to the co-payments that patients pay for PBS medicines. More immediately, the co-payment increase scheduled to take effect on 1 January 2005 provides lessons on how co-payment increases might be handled in the future to enhance sustainability while ensuring the best health outcomes.

5.8.1 2005 co-payment increases

PBS co-payments are scheduled to increase by 21 per cent on 1 January 2005. General patients' co-payments will increase from $23.70 to $28.60; concession card holders' co-payments will increase from $3.80 to $4.60. Medicines Australia supports the co-payment increases and the development of a responsible co-payment system, provided that the health and wellbeing of those that can least afford the increases is not compromised.

Nevertheless, Medicines Australia believes that there is a risk that patients, facing a significant increase in co-payments, will choose not to have their prescription(s) filled, at least in the short-term. The size of the increase scheduled for 1 January 2005 will probably have a disproportionate impact on concession card holders, in particular the poor and elderly, who may respond by not dispensing or delaying prescriptions recommended by their doctor. This could have potentially serious implications for their own health and for the broader effectiveness of the health system, with added stress on the health system. For

these reasons, Medicines Australia suggested to the Government that the proposed 2005 increase could be phased in over a period of time, say one year.

In the future, the Government could consider phasing in co-payment increases to ensure that the increase does not unduly impact on patient health and the efficiency of the health system.

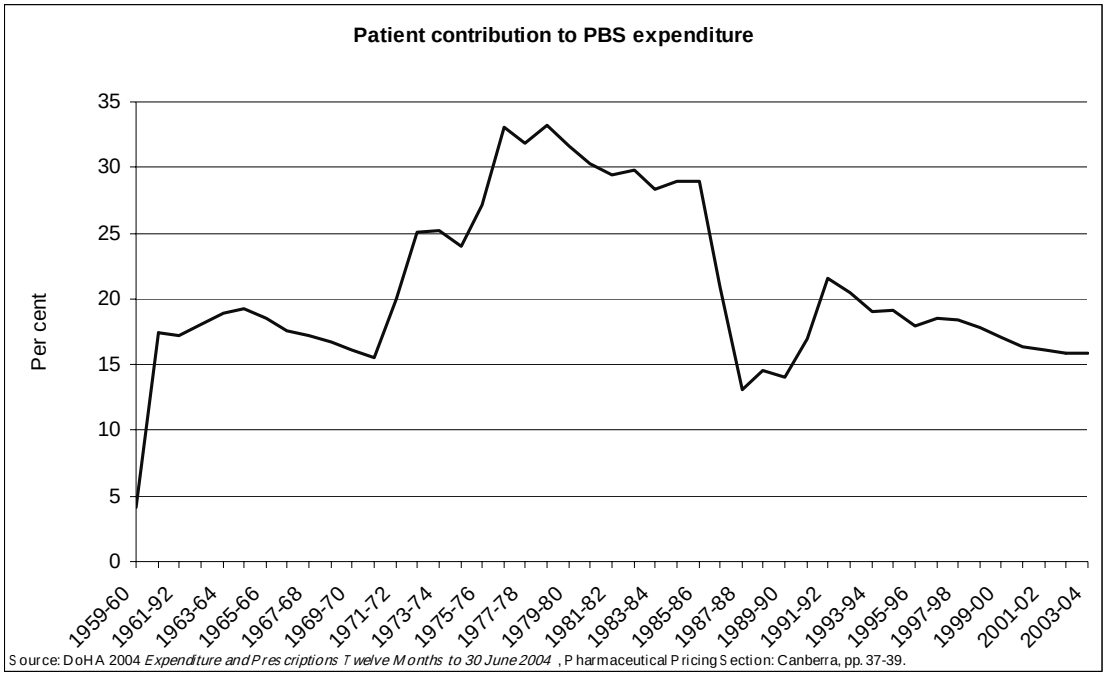
5.8.2 Longer term co-payment options

In the longer term, the Government could consider a range of options with respect to PBS co-payments to ensure a sustainable PBS. Both the flat rate co-payments and fixed Safety Net thresholds are inconsistent with horizontal and vertical equity principles. In other words, high income earners (such as a 'millionaire') are subject to the same co-payment and Safety Net threshold as a person earning less than $30,000 a year with dependent children.

This impact will become starker following the extension of concession cards to self-funded retirees with an annual income of up to $50,000. As a result, a family with one breadwinner on an annual income of, say $30,000, will pay more for medicines than a self-funded retiree on the same or much higher superannuation and investment incomes.

Co-payments are adjusted for movements in CPI. However, the slower growth in co-payments, compared to average medicine costs, and increased demand as reflected in the growth in volumes, has placed a greater cost burden on the Government. Patients' contribution to the PBS has declined from around 30 per cent in the 1970s to just 15.8 per cent in 2003-04 (Figure 9). Thus in times past the contribution by patients to the PBS has been double what it is today.

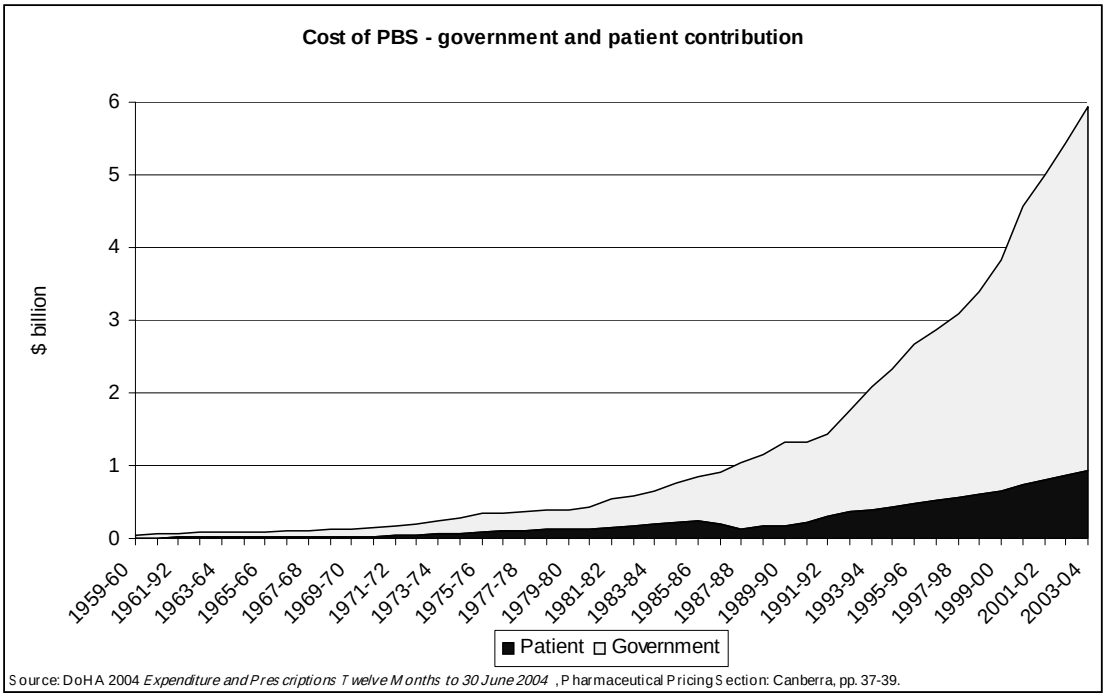
Figure 9



Source: DoHA 2004 *Expenditure and Prescriptions Twelve Months to 30 June 2004* , Pharmaceutical Pricing Section: Canberra, pp. 37-39.

The falling patient contribution is further illustrated when compared with what the Government contributes to the PBS. In nominal terms, while the patient contribution to the PBS has slowly increased to $938 million over the last four decades, the Government's contribution has grown to $5.0 billion (Figure 10). Most of the growth in the PBS over this time has been met by the Government.

Figure 10



In light of the above, there are merits in reviewing the co-payment and safety net policies to address the following issues:

- To differentiate between lifesaving medicines and less essential medicines so that the PBS would give greater support to 'essential' medicines;
- To re-balance the distributional impact between different income groups;
- To ensure that those who can afford to pay assume greater individual responsibility; and
- To provide greater market signals in consumer choice.

Australia is one of few countries to require a flat co-payment. Other countries including France, Belgium, Italy and Denmark, have utilised variable co-payment systems depending on whether medicines are lifesaving or for less severe conditions. This has given these governments the flexibility to target expenditure at national health priority areas and on addressing severe health conditions.

One option could involve patients paying different levels of co-payments based on priority. Co-payments for PBS medicines could vary depending on whether a medicine is life-saving, essential or otherwise. Within each category, co-payments may also be determined by the patient's ability to pay.

Alternatively, co-payments could be based on whether a medicine treats one of the Government's National Health Priority Area (NHPA). For example, medicines that address a NHPA could have a lower co-payment than medicines that do not. This would ensure that the public subsidy provided by the PBS is targeted at those areas most important to the Government's health policy priorities.

Another option is that co-payments could be means-tested. Co-payments could increase with income, such that low income earners pay a lower co-payment than those on high incomes.

Another option could be to have a proportional co-payment where the patient pays a fixed percentage of a medicine's cost, say 10 per cent. This policy would adopt a standard patient contribution to the cost of medicines available on the PBS. However, there should also be a floor (a minimum co-payment level) and a ceiling (a maximum payment level) to ensure no undue hardship or a further reduction in patient contribution to the PBS budget.

These options would transfer some of the burden of the PBS expenditure to the patient and may reduce overall consumption through demonstrating the real cost of medicines to patients. The design of the new co-payment and Safety Net system and the rates of co-payments would need to be targeted carefully to minimise significant impacts on disadvantaged groups.

In the context of the challenges facing the health system, Medicines Australia argues that the Government should develop a comprehensive White Paper on the National Medicines Policy and in particular the future ability of the PBS to deliver affordable medicines when Australians need them. It should examine PBS policy options to ensure the delivery of the desired health outcomes through equitable, timely access of medicines, with an assessment on the impact of these options on other parts of the health system, community expectations and intergenerational equity considerations.

5.9 Other options for funding arrangements

There are other funding options available to ensure that the community has access to the latest innovative medicines. Future sustainability may be served if the community takes greater direct responsibility for funding access to medicines. There may be a need to investigate alternative private sector funding arrangements for access to medicines. Two particular options in this area are medical savings accounts (MSAs) and greater pharmaceutical coverage by private health insurers.

5.9.1 Medical savings accounts

MSAs operate in a similar way to superannuation, whereby people invest some of their savings to fund their future health and pharmaceutical costs as they get older. They could be attached to people's superannuation accounts. Singapore is a notable example where MSAs held by individuals are used to fund a portion of that country's health spending.

5.9.2 Private health insurance

An alternative is to have private health insurance play a greater role in covering the cost of people's prescription medicines. While some health insurers now cover prescription medicines, the overall coverage of medicines in Australia is low, and health insurers are prevented from offering co-payment coverage. Some countries have a greater proportion of their pharmaceutical costs borne by private health insurance. For example, in Canada, private health insurance plans account for around 34 per cent of prescription medicine costs[45]. Private health insurance could potentially play a larger role in Australia in encouraging private funding of prescription medicines in the future, including the funding of individual expenditure in co-payments.

5.10 Need for stakeholder involvement in policy development

The range of issues and complexities influencing future healthcare expenditure mean that the development of any policy initiatives needs to be done in consultation with key stakeholders in the health system. Doctors, the pharmaceuticals industry, pharmacists and consumers all need to be involved in developing policy solutions to secure the sustainability of pharmaceuticals expenditure, such as the PBS, and healthcare spending more generally. These stakeholders need to be involved in all key policy formation and decision making steps. Such collaborative efforts can help identify potential policy options, assess their feasibility and constructively engage those parts of the health system that will implement them and be affected by them.

[45] Commission for the Future of Health Care in Canada 2002 *Building on Values: the Future of Health Care in Canada*, (Commissioner: Roy J. Romanow QC), November, http://www.hc-sc.gc.ca/english/pdf/care/romanow_e.pdf (accessed 24 November 2004), p. 195.

6. ELASTICITY AND THE IMPACT OF PBS CO-PAYMENT INCREASES ON THE USE OF MEDICINES

How developments in new pharmaceutical technologies will affect health care expenditure depends in part on the demand response to policy changes that influence the prices paid by consumers for medicines. In Australia, this principally involves assessing how changes in PBS co-payments impact on the use of medicines. Research in Australia and overseas suggests that there is at least some price elasticity in the use of medicines; that is, that price or co-payment increases impact on use of medicines.

While co-payment increases may be an important part of developing a sustainable health system, the evidence suggests that in the short-term such increases may lead to some members of the community not filling their prescriptions, unless co-payment increases are implemented carefully. This may include, for example, phasing in such increases over a certain period of time, rather than patients encountering a co-payment increase in one step.

6.1 Overall elasticity

Evidence indicates that increasing co-payments levels leads to falls in the use of medicines. There are various studies that examine this in different ways. Certainly not all agree on the exact elasticity; that is, the extent to which an increase in co-payments leads to a fall in the use of medicines. However, they all agree that, in general terms, script volumes fall with increasing co-payments.

The National Centre for Social and Economic Modelling (NATSEM) has studied the likely impact of co-payment increases on script volumes using the MediSim model it developed in partnership with Medicines Australia. NATSEM's study[46] estimates the impact of what would have happened in 2002-03 if co-payments had been increased by 25 per cent. Given that the co-payments will increase by 21 per cent on 1 January 2005, the NATSEM modelling provides a reasonable estimate of the likely impact.

The NATSEM study suggests that a 25 per cent increase in co-payments, for both general and concessional patients, will result in a fall in overall script volumes of 8.3 per cent. In the scenario run with the MediSim model, scripts for 2002-03 were 8.3 per cent lower than the base case. In addition, scripts for the following year, 2003-04 were estimated to be 9 per cent lower than the base year, suggesting that the impact of an increase in co-payments can last two years.

Other studies have looked at the impact of co-payment increases on PBS script volumes. A study by Johnson (1990), estimates that a 10 per cent increase in the PBS co-payments would lead to a fall in script volumes of at least 2.5 per cent[47]. In 1986 the former Bureau of Industry Economics estimated that a 10 per cent reduction in the price of medicines led to an increase in prescriptions of between

[46] NATSEM/Medicines Australia 'MediSim' scenario, 2003.
[47] Quoted in Industry Commission 1996 *The Pharmaceutical Industry*, Volume 1, p. 189-190.

1.7 and 2.5 per cent[48]. An implication from this is that a 10 per cent *increase* in co-payments should result in roughly the same sort of decline in prescriptions.

A British study found that increases in the price of medicines leads to reductions in script volumes. It found from data over the period 1969 to 1986 that a 10 per cent increase in price leads to a 3.3 per cent fall in script volumes, although the decrease was higher at 6.4 per cent in the shorter period 1978 to 1986[49]. This study also found that the demand for over the counter products increased following a rise in prescription medicine prices. Other research has suggested that a 10 per cent rise in medicine prices leads to a fall of one to three per cent in the number of prescriptions[50].

6.2 Differences in therapeutic areas

Moreover, the evidence suggests that the fall in the use of medicines will not be uniform across all therapeutic areas. A study in the Journal of the American Medical Association (JAMA) earlier this year[51] found that doubling co-payments (a 100 per cent increase) was associated with reductions in the use of eight therapeutic classes. Falls were measured in reductions in overall days supplied for non-steroidal anti-inflammatories (NSAIDs) (45 per cent), antihistamines (44 per cent), antihyperlipidemics (34 per cent), antiulcerants (33 per cent), antiasthmatics (32 per cent), antihypertensives (26 per cent), antidepressants (26 per cent) and antidiabetics (25 per cent).

The same study found that the use of medicines by patients with chronic conditions was less responsive to a doubling of co-payments. However, some therapies still saw a fall in use by chronic patients. The use of antidepressants by depressed patients still declined 8 per cent, use of antihypertensives by hypertensive patients fell by 10 per cent. Stronger effects were observed in other conditions, where arthritis patients' use of NSAIDs fell by 27 per cent, allergy patients cut their use of antihistamines by 31 per cent and diabetics reduced their use of antidiabetes medicines by 23 per cent. This suggests that even patients who perhaps should be taking medication for particular conditions reduce their use of medications with co-payment increases.

A study reviewing the international experience of increases in the cost of medicines found that both essential and discretionary medications saw a fall in usage[52], with a further study suggesting that discretionary medications are likely to see greater falls in use than essential medicines[53]. Recent research by the RAND Corporation also shows the differential impact of co-payment increases on

[48] Ibid, p. 189.
[49] O'Brien, Bernie 1989 "The Effect of Patient Charges on the Utilisation of Prescription Medicines" *Journal of Health Economics*, v. 8, pp. 109-132.
[50] Jacobzone, S. 2000 *Pharmaceutical Policies in OECD Countries: Reconciling Social and Industrial Goals*, Labour Market and Social Policy Occasional Papers, No. 40, OECD: Paris, p. 15.
[51] Goldman, D.; Joyce, G.; Escarce, J.; Pace, J.; Solomon, M.; Laouri, M.; Landsman, P. & Teutsch, S. 2004 "Pharmacy Benefits and the Use of Drugs by the Chronically Ill" *JAMA*, v. 291, n. 19, pp. 2344-2350.
[52] Lexchin, Joel & Grootendorst, Paul 2004 "The Effects of Prescription Drug User Fees on Drug and Health Services Use and Health Status: a Review of the Evidence" *International Journal of Health Services*, 34(1), pp. 101-122.
[53] McManus, Peter; Donnelly, Neil; Henry, David; Hall, Wayne; Primrose, John & Linder, Julie 1996 "Prescription Drug Utilization Following Patient Co-Payment Changes in Australia" *Pharmacoepidemiology and Drug Safety*, v. 5, pp. 385-392.

therapeutic areas[54]. It found that doubling co-payments led to falls in use of medicines for depression (8 per cent fall), hypertension (10 per cent), GERD (17 per cent), asthma (22 per cent), diabetes (23 per cent), arthritis (27 per cent) and allergic rhinitis (31 per cent). The same study also found that as a result of the decline in use of medicines, hospital admissions rose 10 per cent and emergency room visits rose 17 per cent.

6.3 Summary of internal research of Medicines Australia member companies

Internal research provided by several member companies of Medicines Australia indicates similar results to that identified in the international literature. In general terms, research provided by several member companies suggests that:

- A 10 per cent increase in co-payments will roughly lead to around 2.5 to 3 per cent fall in overall script volumes in the short term;
- The relationship between price increase and prescription decline tends to be linear;
- Falls in script volumes will not be uniform across the therapeutic areas and will tend to be higher for some therapeutic categories than others, with medications for asymptomatic conditions perhaps having greater falls than medications for symptomatic conditions; and
- The proposed 21 per cent increase in co-payments to occur on 1 January 2005 will lead to an overall reduction in PBS script volumes of around 5 to 7 per cent in 2005.

6.4 Previous increases in PBS co-payments

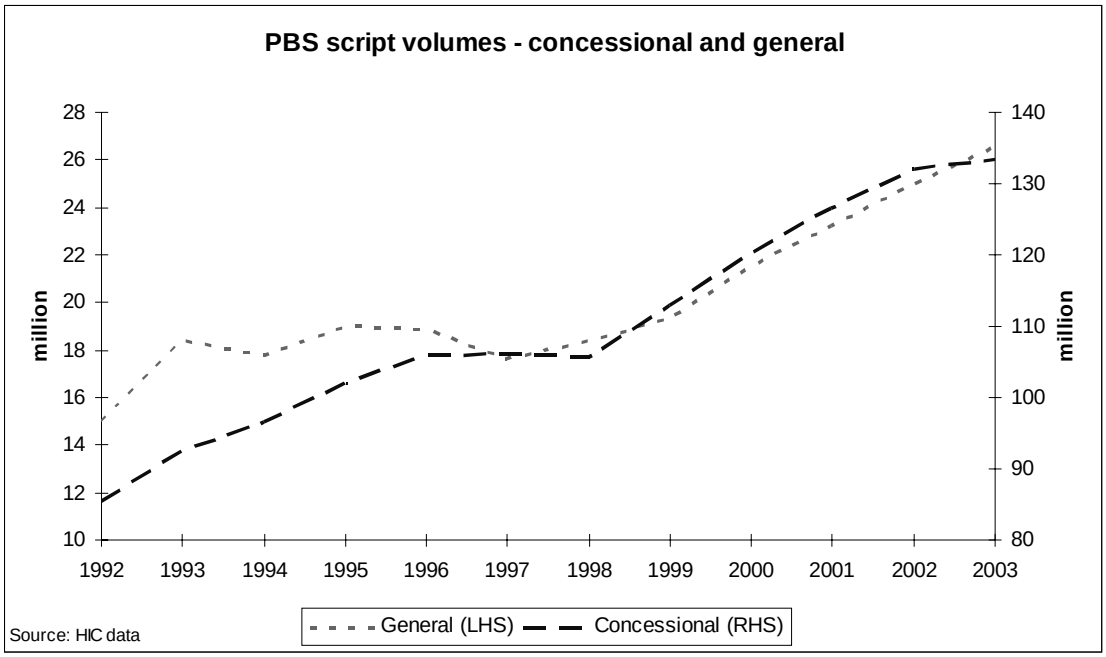
The impact of the 1997 co-payment increases also provides some indication of the likely impact of the proposed 2005 increases. On 1 January 1997, co-payments were increased by 15 per cent for general patients and 18.5 per cent for concessional patients. In that year total PBS script volumes were 0.8 per cent lower than 1996, or 5 per cent lower than would otherwise have been the case [55] for 1997 (HIC data). In 1998 script volumes still only grew by 0.3 per cent, well below the average growth rate for PBS scripts, and were 8.6 per cent below what would have been expected. This supports the NATSEM conclusion that the impact of co-payment increases extends over two years.

In 1997, ordinary general patient scripts (excluding safety net general patients) fell by 8.5 per cent compared with 1996 (HIC data). Scripts for ordinary concessional patient scripts (excluding safety net concessional patients) fell by 0.2 per cent in both 1997 and in 1998 compared with 1996 (HIC data). The falls in two successive years for concessional scripts again demonstrate that the impact of co-payment increases could be felt in the year of the co-payment increase, and the subsequent year.

[54] Quoted in V. Fuhrmans. "Higher co-pays may take toll on health". *Wall Street Journal* 19 May 2004 http://online.wsj.com/article_print/0,,SB108491551729714914,00.html (accessed 20 May 2004).

[55] Five per cent lower than forecast is based on the average annual growth rate of scripts from 1992 to 2003 of 4.3 per cent per annum (HIC data).

Figure 11



Australian research also highlights that script volumes fell as a result of the co-payment increases introduced in November 1990. General co-payments were increased from $11 to $15 at that time, while concessional patients had a $2.50 co-payment introduced where previously they had none. In the calendar year following the co-payment increase, 1991, the number of subsidised prescriptions on the PBS fell by 15.6 per cent[56].

The 1990 increase led to 'discretionary' medicines experiencing larger falls than 'essential' medicines[57]. Here, essential medicines are those treating chronic conditions, the withdrawal of which would have serious consequences, while discretionary are those more related to treating symptoms. This result would appear somewhat at odds with the suggestion of one member company of Medicines Australia that symptomatic conditions might not experience as much of a decline as asymptomatic conditions. The study by McManus et al[58] confirms the point that not all therapeutic classes face an equal decline in prescriptions. This study also found that in 1990 there was a large increase in scripts in the month leading up to the co-payment increase[59], as patients filled scripts before the change came into effect.

6.5 Impacts on different groups in the community

Lexchin and Grootendorst[60], following a widespread review of the international literature, found that it was the elderly and low-income members of the

[56] McManus et al, op cit, p. 390.
[57] McManus et al found that 'discretionary' medicines were 25 per cent lower than would otherwise have been the case, compared to 18 per cent lower for 'essential' medicines, excluding the change in the underlying trend following the co-payment increase. With an unadjusted mean level following the co-payment increase, discretionary medicines were 23 per cent lower. Essential medicines were only three per cent lower, although the authors point out that this last result was "confounded with the apparent secular increase in essential prescriptions over time" (p. 389).
[58] Ibid.
[59] Ibid, p. 389.
[60] Lexchin & Grootendorst, op cit.

community who were the most affected by increases in co-payments. The degree of price sensitivity tends to be higher the larger the share of income spent on prescription medicines.

The same study also identified that people with a lower health status – with two or more chronic health conditions – had a lower elasticity of demand. This means that sicker people did not curtail their demand for prescriptions as much as the general population. They cite a study from Ontario in Canada showing that for such patients, a 10 per cent increase in the price of medicines leads to a fall in demand of between 1.1 and 1.3 per cent[61]. While a lower elasticity, this corroborates the finding of the JAMA study that even sick people will reduce their use of medicines in the face of co-payment increases. The experience of the 1997 co-payment increase shows that the impact on demand by concessional and general patients varies. At that time, general patients experienced a shorter, one off drop in prescriptions, while script volumes for concessional patients showed very slight falls in prescriptions, but over two consecutive years.

6.6 Case study of PBS elasticity: likely impact of the January 2005 PBS co-payment increase

While by no means definitive, the evidence above suggests that the co-payment increase scheduled for 1 January 2005 will reduce the use of medicines. While estimates vary considerably, the balance of opinion from research seems to be that, generally, a 10 per cent increase in co-payments leads to a fall of around 2.5 to 3 per cent in PBS script volumes.

This would suggest that the proposed 21 per cent increase introduced on 1 January 2005 for both concessional and general co-payments would lead to a fall of around 5 to 6 per cent in PBS script volumes in 2005 across the board. However, some therapeutic areas are likely to experience larger falls in script volumes, including in chronic conditions. Moreover, any co-payment increase in 2005 could have a follow-on impact into 2006, possibly of a similar magnitude. The experience of the 1997 co-payment increases, combined with feedback from Medicines Australia member companies and NATSEM results, would suggest that the impact of the co-payment increase could be felt for two years, at least in concessional patients.

International research confirms that it is usually the low income and the elderly that are most affected. As discussed in the preceding section, such a fall could have significant adverse implications for the health of Australians and the quality use of medicines. The lack of available data on the impact of co-payment increases in Australia suggests that the after effects of the proposed increases in 2005 should be monitored to assess their impacts on the PBS, the community, different patient groups and, ideally, health outcomes.

[61] Ibid.

7. OTHER DRIVERS OF DEMAND FOR PHARMACEUTICAL TECHNOLOGIES

The demand for new medical technology, such as innovative medicines, is driven by a range of additional factors in addition to price. Government policies and priorities influence the level and mix of technologies and therapies introduced, particularly in the case of pharmaceuticals given the importance of the PBS in Australia. Greater information about new treatments, such as from new research and company information, provides greater options to health practitioners. Prescriber behaviour and the management of that also plays a significant role in influencing the demand for new technologies. Consumer expectations also play a significant role in the demand for new technologies – patients want access to the newest treatments for illness. All of these factors influence the demand for new health technology and therefore the level of expenditure on these technologies. This section looks at how these factors influence innovative medicines, a major area of health technology.

7.1 Health policy and the National Health Priority Areas

Government policy is one of the major drivers of demand for innovative medicines in Australia. Two main areas of health policy that are relevant to a discussion of demand for pharmaceutical technologies are:

- The National Medicines Policy; and
- National Health Priority Areas.

The National Medicines Policy ties a number of related strategies and objectives together. It is a partnership between the Commonwealth, States and Territories, the various stakeholders in the health sector, consumers and the media and is designed to promote positive health outcomes through the appropriate access and use of medicines. The four pillars of the National Medicines Policy are:

- Timely access to the medicines that Australians need, at a cost individuals and the community can afford;
- Medicines meeting appropriate standards of quality, safety and efficacy;
- Quality Use of Medicines; and
- Maintaining a responsible and viable industry.

Some of these elements are related to demand for pharmaceutical technology and these are addressed below.

As the name suggests, Australia's National Health Priority Areas are the health conditions that the Commonwealth, State and Territory governments have agreed to work together to achieve better health outcomes. Australia currently has seven National Health Priority Areas:

- Cardiovascular disease;
- Asthma;
- Cancer;
- Diabetes;
- Mental health and suicide prevention;
- Accident and injury prevention; and
- Musculoskeletal disease.

An eighth, Dementia, was announced by the Government during the 2004 Federal election campaign.

Medicines clearly play a major role in most of these areas with the possible exception of accident and injury prevention (although medication prescribing or administration errors and related medicine misadventures are a significant problem in Australia and the subject of considerable research and strategy development in the medical quality and safety area). As discussed in Section 5, there are 579 new medicines currently in clinical trials that address Australia's National Health Priority Areas, although not all of these will make it to market.

For the main chronic illness areas such as diabetes, asthma, cardiovascular disease, musculoskeletal disease and mental health, medicines are central to improved outcomes in the past 10-20 years. The introduction of statin medications for elevated cholesterol, improved oral hypoglycaemic medications for type 2 diabetes, inhaled corticosteroids for asthma and SSRI antidepressants for depression have all had significant impact on disease burden.

To understand how policies and strategies across disease areas impact on technology use and overall cost, it is useful to consider how costs change in specific health areas. In general, increases in health costs can be attributed to either *increasing cost per treated case* or *increased treated prevalence*.

In the former, technological advances often change the way in which a particular episode or diagnosis is treated. Cardiovascular disease is a good example of increased cost per treated case. While the prevalence of cardiovascular disease is not increasing (due to the effect of public health initiatives, increased awareness of importance of diet and exercise, and widespread use of cholesterol lowering medication), the cost per treated case has increased as new, higher cost technologies are widely adopted.

For example, the advent of cardiac imaging technologies, the use of cardiac stents and medications that enhance revascularisation outcomes by inhibiting repeat clot formation and stenosis have become first line practice for cardiac ischaemic events and are relatively high cost.

In the latter, there are some conditions that are characterised by increased community awareness and/or improved diagnostic capabilities. Depression is a good example of a condition that appears to be both increasing in prevalence because it is also increasingly diagnosed due to changes in public awareness and reduction in stigma. Diabetes is another example where the overall prevalence continues to increase at an alarming rate. Both these conditions are examples where the cost per treated case has not risen significantly over recent years, but the overall cost of the illness has increased significantly. In these conditions, while overall costs are increasing, technology is not driving up the cost but is improving outcomes.

Accordingly, if the policies and strategies associated with Australia's National Health Priority Areas involve either increased adoption of proven technologies or increased awareness and diagnosis, then the overall contribution of technology to total health costs will continue to increase, but health outcomes will improve. The better outcomes from the newer technology are also directly addressing priorities set in government policy. Government policy is therefore a key driver of demand for new health technologies and subsequent health expenditure.

7.2 Research findings

Patients and prescribers can become aware of, and gain experience of, new medications through the results of clinical trials. From time to time major clinical trials are conducted with a scale and significance that clearly influences how technologies are taken up. This has been most apparent in the cardiovascular area, in part because the high prevalence of cardiovascular disorders in all developed countries makes it possible to recruit very large numbers of subjects. Over the past ten years several very large studies have demonstrated unequivocally the benefits of statin medications in reducing the risk of ischaemic cardiovascular events. This has contributed to prescriber and patient uptake of medicines.

For example, earlier this year the journal Lancet published a 20,000 subject, five year study of simvastatin. The subjects were aged 40 to 80 and considered at high risk of stroke. Compared to a placebo, simvastatin reduced the risk of stroke by 25 per cent. The results of this study have highlighted the benefits of these medicines to patients and doctors. Studies such as this have a considerable influence on the utilisation of medical technologies including pharmaceuticals, via the shaping of formal guidelines and as evidence for additional health technology assessment such as the PBAC process.

7.2 Prescriber behaviour and incentives

Prescriber behaviour is a key driver of demand for new, innovative medicines. Doctors want to have access to the latest technologies for use with their patients.

Doctors in Australia exercise a broad measure of prescribing freedom. General Practitioners' prescribing habits are regarded as an important contributor to current rates of increase in the national medicines bill. GP prescription forms contain a box that doctors may tick in order to forbid generic substitution by pharmacists, but few GPs make use of this option. Equally, however, few prescribe generically and generic prescribing is not widespread.

Initiatives designed to encourage more rational prescribing behaviour among the medical profession have emerged at both local and national level. Prescribing guidelines have been developed in a range of therapeutic areas, but are not strict. They focus on the identification of target populations and appropriate prescribing rather than on restrictive goals, such as the use of cheaper alternative medicines or lowering prescription volumes.

The establishment of the National Prescribing Service (NPS) was announced in May 1997. This is an independent body whose aim is to support 'quality use of

medicines' initiatives in key therapeutic areas through the development of education programmes. Both the pharmaceutical industry and the medical profession are represented on the NPS board.

One key objective of the NPS is the development of national treatment guidelines and protocols, which will begin to emerge in key therapeutic areas over the next five years. NPS prescribing guidelines have already been issued for NSAIDs and antibiotics. The NPS provides prescribing feedback and offers educational and decision-making support for individual doctors whose prescribing activity is at variance with other physicians in a particular area.

The practice behaviour of Australian GPs is shaped in a number of other ways. One of the most successful examples of the use of financial incentives in the form of practice incentive payments was the Commonwealth program to increase childhood vaccination rates. Another more recent example is the 3+ Asthma plan, in which GPs receive extra payments for a series of patient visits to ensure the patient's asthma is fully controlled. Recently, the Government introduced the Better Outcomes in Mental Health initiative, in which GPs receive additional payments for undertaking further education and training in mental health, and partner with other health professionals in delivering improved care.

While these incentive programs are not directly targeted at prescribing, if pharmaceuticals form a part of the best practice being encouraged their use will of course increase.

7.3 Consumer expectations

Consumers are increasingly taking more control of the decisions and actions related to their health. They are also accessing more information about the variety of treatment options available to them. The role of consumers in regard to prescribing decisions varies. Some consumers have a dislike of their GP 'reaching for the prescription pad'. This applies particularly for some conditions where consumers would rather receive non-drug therapies if available (eg depression). In contrast, in other conditions such as high cholesterol, many consumers would rather just 'take a pill' rather than undertake lifestyle changes such as improved diet or increased exercise.

In other situations such as diabetes and cancer, consumers and carers are becoming well informed via the Internet and networks of support organisations. New technologies become widely known very quickly and Australian doctors are often faced with requests for therapies not yet readily available locally.

7.4 Industry activity

Clearly, a major influencer on the take-up of new medicines is the pharmaceutical industry itself. Having invested between US$750 million and US$1 billion to develop a new medicine from 'bench to bedside', a pharmaceutical company will seek to enter the market to obtain a return for that investment. However, there is increasing recognition that this requires balanced information and collaboration with a range of stakeholders. The industry continues to rely heavily on 'medical representatives' to promote products face to face with prescribers. Increasingly,

the additional marketing activities undertaken by the industry are becoming aligned (both in content and delivery) with broader educational programs involving other partners such as the specialist colleges. As part of a Federal Budget initiative in 2003, the Australian pharmaceutical industry agreed to highlight details of any listing on the Pharmaceutical Benefits Scheme in any promotional materials, in an effort to ensure prescribers acted more in line with PBS listings.

The area of direct-to-consumer advertising of pharmaceuticals continues to be controversial globally. The United States and New Zealand are currently the only jurisdictions where direct to consumer advertising is permitted. In Australia advertising direct to consumers by industry is prohibited. The industry adheres to this legislation and recognises recent statements in the United States-Australia free trade agreement as a reaffirmation of existing policy.

At the same time, industry sees a role for increased information on medicines being made available to consumers to encourage informed dialogue with their health professionals. For example, industry members have funded public health related promotions focused on areas such osteoporosis and diabetes where epidemiological studies have confirmed that many people with these conditions go unrecognised because they may be asymptomatic in the initial stages of disease. Similarly, some companies have funded advertisements on erectile dysfunction because market research in Australia shows neither consumers nor GPs are comfortable discussing this topic and therefore consumers who could benefit from an intervention may not receive that benefit.

7.6 Factors capable of modifying demand

In addition to the above, there are several factors that may modify demand, either directly or indirectly.

7.6.1 Quality use of Medicines

Quality Use of Medicines (QUM) is one pillar of the National Medicines Policy. QUM implies understanding of the treatment options for a condition (pharmacological as well as non-pharmacological) and an understanding of the elements to consider when choosing between medicine options.

Properly implemented, QUM programs can contribute to all stakeholders being aligned regarding the optimal place for a new medicine. Clearly, if consumers have been exposed to similar information that GPs have been (for example, with regard to relative and absolute risk, number needed to treat, and overall risks and benefits) then there will be greater alignment around the decision to prescribe (or not to prescribe).

This year the Chairman of PBAC, Professor Lloyd Sansom, requested the PHARM Committee (the Ministerial advisory committee on QUM) to investigate options for incorporating QUM into the guidelines and submission processes for PBAC. This work is progressing and may prove to be very important in ensuring increased understanding of, and commitment to, QUM principles by the industry.

7.6.2 Independent information sources

Critics of industry claim that there is an imbalance of information, with biased information being provided to health professionals by the industry and little available to 'counteract' that information. However, without such marketing activity, doctors of would be deprived of much information on new medicines. In addition, the industry's medical representatives are largely tertiary trained professionals who complete a five-part industry training program as part of their role and adhere strictly to the industry's Code of Conduct. Both the training program and the Code of Conduct are supported by Medicines Australia.

From its early beginnings, the National Prescribing Service has expanded to become a substantial organisation with field workers delivering guidance to general practitioners via the Australian Divisions of General Practice network, regular continuing education and audit activity and, more recently, consumer QUM activity.

More recently, the Rational Assessment of Drugs and Research (RADAR) program was introduced specifically to provide early information on new medicines at the time of PBS listing. This has required NPS to have a presence at PBAC meetings and for NPS to be provided with confidential information presented to the PBAC by the sponsor company, along with documentation from the PBAC evaluation process. The NPS is required to work with the sponsor company in developing the information for the RADAR program.

7.6.3 Access to specialist services

Some pharmaceutical technologies may require either specialist prescribers or the application of specialist diagnostic or other technologies in order to be prescribed.

For example, if a new treatment for Attention Deficit Hyperactivity Disorder is to be listed on the PBS, it is likely to require prescription by a specialist (either a child psychiatrist or a behavioural paediatrician) at least for the initial prescription. The geographical distribution and availability of such specialists means there may be an automatic restriction on the rate of uptake for a new technology, regardless of the clinical need in some areas.

Another example is the potential listing on the PBS of antiresorptive medications (that increase bone mineral density) to prevent fractures in men or women with a combination of low bone density and advancing age. The take-up of antiresorptive medications under such a listing is obviously dependant on the access to specialised bone density measurement facilities, a technology usually only available in specialised clinics attached to academic endocrinology units.

8. THE POTENTIAL FOR MEDICINES TO OFFSET COSTS IN OTHER PARTS OF THE HEALTH SYSTEM

8.1 Newer, innovative medicines can reduce overall health costs

As well as their broader social, economic and health benefits, spending on medicines can also provide offsetting savings in other parts of the health system. Illnesses that once required expensive hospitalisation, nursing homes or surgery such as diabetes, heart attacks, depression and schizophrenia, are now increasingly being treated by new medicines developed by the pharmaceutical industry. In the future, conditions like Alzheimer's Disease, which currently cost the community much in terms of residential care, family and carer costs, may be treatable with new medicines currently being developed. While in some cases the newer generation of medicines themselves are more expensive, in many cases this is more than offset by falling costs in other parts of the health system.

For example, the four most costly products listed on the PBS (see Section 2) all help to reduce costs in other parts of the health system. Atorvastatin and simvastatin, the two most costly products, help reduce the risk of heart attacks and strokes which are treated in hospitals. Similarly, omeprazole assists in healing stomach ulcers, reducing the need for surgery, and salmeterol/fluticasone helps manage asthma which also reduces hospital costs.

Unfortunately, broader Government policy does not seem to recognise the impact that new, innovative medicines can have by reducing cost pressures in other parts of the health system. The overriding concern in the 2002 Intergenerational Report and the more recent Australia's Demographic Challenges documents is that spending on the PBS is simply a cost. The Government's additional concern is the cost of the PBS is growing and that measures must be taken to curtail that growth.

There is insufficient acknowledgement in these statements that such pharmaceutical spending can, in fact, help reduce overall health expenditure. There is a growing body of evidence to suggest that increased spending on medicines can and does lead to greater offsetting savings in other parts of the health system. Treating conditions like high cholesterol, mental illness and cancer with medicines now can reduce the need for more expensive options such as hospitalisation and surgery.

The result is savings in other parts of the health system. The Chair of the Productivity Commission has flagged the fact that spending on medicines could give rise to savings in other parts of the health system[62]. However, the Intergenerational Report makes no allowance for the interactions between different parts of the health system like the PBS and hospital costs[63].

[62] Banks, G. 2004 "An Ageing Australia: Small Beer or Big Bucks?" *Presentation to the South Australian Centre for Economic Studies, Economic Briefing*, 29 April: Adelaide, p. 24.
[63] Dowrick, S. & McDonald, P. 2002 "Comments on Intergenerational Report, 2002-03", Australian National University: Canberra, p. 10.

Medicines Australia strongly argues that any future consideration of the effects of ageing on the PBS should consider the positive impacts that spending on medicines has on both workforce productivity and participation, and the potential for such spending to provide offsetting savings in other parts of the health system.

Most studies of cost offsets are from other countries where health systems have linked data sets, such as in the US managed care environments. Unfortunately, few equivalent studies are available at this point in Australia due to a lack of data linking patient use of hospitals, medical services and pharmaceuticals.

8.2 International evidence

International research suggests that a general increase in spending on medicines is more than offset by greater savings in other parts of the health system. A 1996 study by Lichtenberg in the *American Economic Review* found that for every US$1 increase in spending on medicines there was a US$3.65 saving in hospital care expenditure[64].

Freund and Smeeding in their discussion of future health care costs in an ageing society point out that governments often do not take the benefits of spending on medicines into account. *"By far, the most important lesson to be learned here is that governments and policy analysts consider only the costs of new treatments and new medicines, and ignore the benefits"*[65]*.* Making a full assessment of the impact of medicines can only be made once the benefits of those medicines, both for productivity and for other costs in the health system, are taken into consideration. Nobel laureate, Professor Gary Becker, makes the point that new medicines can potentially cut overall health costs.

"The share of drugs in future medical spending is likely to increase sharply. But even without full cures, drugs that greatly delay the onset and severity of major diseases will reduce expensive and unproductive time spent in hospitals, nursing homes, and under the care of family members … New drugs have the potential to cut the growth of medical spending sharply. It is crucial to take much better advantage of this potential"[66].

In his review of studies into the impact of rising medicine costs on overall health budgets, Kleinke concluded that new, more expensive medicines save costs in other parts of the health sector. The shift to more capital-intensive forms of treatment gives rise to increased efficiencies and represents the health sector moving towards the 'new economy'.

"In the aggregate and in the short term, 'expensive' new drug technologies are a bargain for society. Increased spending on drugs that specifically manage disease, preclude or delay surgeries, or reduce hospital admissions and lengths-

[64] Lichtenberg, F. 1996 "Do (More and Better) Drugs Keep People Out of Hospitals?," *American Economic Review* 86, May, 1996, 384-388.
[65] Freund, D. & Smeeding, T. 2002 "The Future Costs of Health Care in Aging Societies: Is the Glass Half Full or Half Empty?" Prepared for the Seminar *Ageing Societies: Responding to the Policy Challenges*, 8 April, University of New South Wales, p. 18.
[66] Becker, G. "New Drugs Cut Costs, And Medicare Can Help", *Business Week*, 22/3/04, p. 32.

of-stay pay for themselves many times over. Added pharmacy costs that offset other medical costs represent the economics of progress. They reflect a profound, permanent movement in our health care system away from medical labor and toward medical technology - a belated catching-up of health care with the rest of the 'new economy'. The added costs associated with breakthrough drugs represent a major structural shift from the provision of traditional medical services to the consumption of medical products, a systemic rotation from labor to capital"[67].

The change from expensive, labour-intensive health treatments such as hospitalisation and surgery, in favour of capital-intensive treatments such as medicines is a major structural shift in healthcare towards a more efficient kind of health expenditure.

Kleinke's review highlights several studies that identified the effect of how the switch to more capital-intensive treatments in using newer, innovative medicines have reduced the overall costs of treating HIV/AIDS and psychiatric illness. This occurred because the increased cost of new, more effective medicines was more than offset by falls in hospitalisation rates[68]. Other studies found that restricting the re-imbursement of three medicines in the US Medicaid program *"increased the rates of institutionalization in nursing homes, emergency mental health visits, and full-day or half-day hospitalizations in community mental health centers - all at costs far in excess of the medicine savings"*[69].

The Value of Medicines: Longer and Better Lives, Lower Health Care Spending, A Stronger Economy

Over the last few decades, scientists have made substantial progress in discovery of new medicines. Even more dramatic advances are anticipated in the years ahead through research in new fields such as genomics and proteomics.

In the last decade alone, over 300 new medicines have been approved by FDA. These advances are improving the treatment of common diseases like heart disease, diabetes and cancer, as well as rare disorders like Fabry's disease, cystic fibrosis and sickle cell anemia.

As a result of these new discoveries, medicines are taking on an increasingly important role in patient care. As a result, we are spending more on pharmaceuticals. In return, more patients are living longer, better lives; overall health care costs are restrained as patients avoid invasive surgeries and costly hospital and nursing home stays; and the economy is strengthened through improved worker productivity.

A growing number of studies are confirming the increasing value of new medicines to patients and society. For example, a study by Frank R. Lichtenberg, the Courtney C. Brown Professor of Business at Columbia University, finds that patients using newer drugs were significantly less likely to die and lose workdays than those using older drugs. Lichtenberg also found that the use of newer medicines increased drug costs by US$18, but reduced hospital and other non-drug costs by US$129,[i] meaning that for each additional US$1 spent on newer pharmaceuticals, US$6.17 is saved in total health care spending, US$4.44 of which comes from savings in hospital spending.

[67] Kleinke, J. 2001 "The Price of Progress: Prescription Drugs in the Health Care Market", *Health Affairs*, 20(5), Sept-Oct.
[68] Ibid.
[69] Ibid.

New Medicines Save and Improve Lives

New medicines have made a major contribution to the decline in the death rate from HIV/AIDS in the U.S. over the last 10 years. Since the mid-1990s, when researchers developed a new wave of medicines to treat HIV/AIDS, the U.S. death rate from AIDS dropped about 70 percent.[ii]

Several studies have found that use of statin therapy to treat people with high cholesterol reduces hospital admissions and invasive cardiac surgeries. For example, a study of one statin showed that it reduced hospital admissions by a third during five years of treatment. It also reduced the number of days that patients had to spend in the hospital when they were admitted, and reduced the need for bypass surgery and angioplasty.[iii]

A study sponsored by the Agency for Health Care Policy and Research concluded that increased use of a blood-thinning drug would prevent 40,000 strokes a year, saving US$600 million annually.[iv]

A February 2004 study by Lichtenberg finds that new cancer drugs have accounted for 50-60 per cent of the gains we have made in cancer survival rates since 1975. Since 1971, when the U.S. declared war on cancer, our arsenal of cancer medicines has tripled. During that time, the survival rate rose from 50 per cent to 62.7 per cent. Overall, new cancer drugs have contributed a remarkable 10.7 per cent of the increase in life expectancy at birth in the U.S.[v]

New Medicines Help Control Health Care Costs

A January 2004 study by Duke researchers found that "beta-blocker therapy improves the clinical outcomes of heart failure patients and is cost saving to society and Medicare." The study, which was written before enactment of the Medicare drug benefit, notes: "If medication costs were completely reimbursed by Medicare, program savings from beta-blocker therapy would remain positive."[vi]Looking at the overall societal perspective, the researchers found that five years of treatment for heart failure without beta-blockers cost a total of US$52,999. With beta-blockers added to treatment, total treatment costs fell by US$3,959, patient survival increased by an average of about three-and-a-half months, and patients needed fewer overnight hospital stays.

New studies are showing how newer, better medicines reduce the cost of treating people with depression. "The cost of treating a depressed person fell throughout the 1990s, largely because of a switch from hospitalization to medication," the Wall Street Journal said in a December 31, 2003, story on the study. The study, published in the Journal of Clinical Psychiatry in December 2003, found that per-patient spending on depression fell by 19 per cent over the course of the decade[vii].

New diabetes medicines are helping patients avoiding serious complications and death, and can reduce overall health care spending. One recent study found that effective treatment of diabetes with medicines and other therapy yields annual health care savings of US$685 - US$950 per patient within one to two years.[viii] Another study corroborated these results, finding that use of a disease management program to control diabetes with medicines and patient education generated savings of US$747 per patient per year.[ix]

A study of the effects of a new Alzheimer's medicine, donepezil, on costs in a Medicare managed care plan showed that, although the prescription costs for the group receiving the drug were over US$1,000 higher per patient, the overall medical costs fell to US$8,056 compared with US$11,947 for the group not receiving drug treatment. This one-third savings was the result of reduced costs in other areas, such as hospital and skilled nursing facility costs.[x]

New Medicines Strengthen the Economy
New medicines also benefit the economy by increasing worker productivity and reducing absenteeism. One study, which evaluated the effect of migraine treatment on productivity, found that more than 50 per cent of workers who received a triptan drug injection for a migraine attack returned to work within two hours, compared with 9 per cent of workers who received a placebo.[xi]

A study in the Journal of Occupational and Environmental Medicine found that patients taking a non-sedating antihistamine for allergies experienced a 5.2 per cent increase in daily work output in the three days after receiving the medication, compared with a 7.8 per cent reduction in work output for workers receiving sedating antihistamines.[xii]

The National Committee for Quality Assurance says that "if every American with depression received care from a health plan or provider that was performing at the 90th percentile level, employers would recover up to 8.8 million absentee days a year."[xiii] NCQA also reported that only 40.1 percent of patients with depression "received effective continuation phase treatment."

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[i] Frank R. Lichtenberg, "Benefits and Costs of Newer Drugs: An Update," (Cambridge, MA: National Bureau of Economic Research, June 2002).
[ii]CASCADE Collaboration, "Determinants of Survival Following HIV-1 seroconversion after introduction of HAART," The Lancet, 362 (2003):1267-1274
[iii] "Cholesterol Pill Linked to Lower Hospital Costs," The New York Times, March 27, 1995.
[iv] D. B. Matchar, G. P.Samsa, Secondary and Tertiary Prevention of Stroke, Patient Outcomes Research Team (PORT) Final Report - Phase 1, AHRQ Pub. No. 00-N001, Rockbille, MD: Agency for Healthcare Research and Quality, June 2000.
[v] Frank R. Lichtenberg, "The Expanding Pharmaceutical Arsenal in the War on Cancer," National Bureau of Economic Research Working Paper No. 10328 (Cambridge, MA: NBER, February 2004).
[vi]PA Cowper, et al., "Economic Effects of Beta-Blocker Therapy in Patients with Heart Failure," The American Journal of Medicine, 116 (2004): 2, 104-111.
[vii] PE Greenberg, et al., "The Economic Burden of Depression in the United States: How Did It Change Between 1990 and 2000?" Journal of Clinical Psychiatry, 64 (2003): 1465-1475.
[viii]E.H. Wagner, et al., "Effect of Improved Glycemic Control on Health Care Costs and Utilization," Journal of the American Medical Association, 285 (2001): 2, 182-189.
[ix]J. Berger, et al., "Economic Impact of a Diabetes Disease Management Program in a Self-Insured Health Plan: Early Results," Disease Management, 4 (2001): 2, 65-73.
[x]JW Hill, et al. "The Effect of Donepezil Therapy on Health Costs in a Managed Care Plan," Managed Care Interface, (March 2002): 63-70.
[xi]R.C. Cady, et al., "Sumatriptan Injection Reduces Productivity Loss During a Migraine Attack: Results of a Double-Blind, Placebo-Controlled Trial," Archives of Internal Medicine,158 (11 May 1998).
[xii]I.M. Cockburn, et al., "Loss of Work Productivity Due to Illness and Medical Treatment," Journal of Occupational and Environmental Medicine, 41 (1999): 11, 948-953.
[xiii]National Committee for Quality Assurance, State of Health Care Quality: 2002 (Washington, DC: NCQA, 2003).

Source: Adapted from PhRMA, http://www.phrma.org/publications/policy/24.04.2004.983.cfm (accessed 3/12/04).

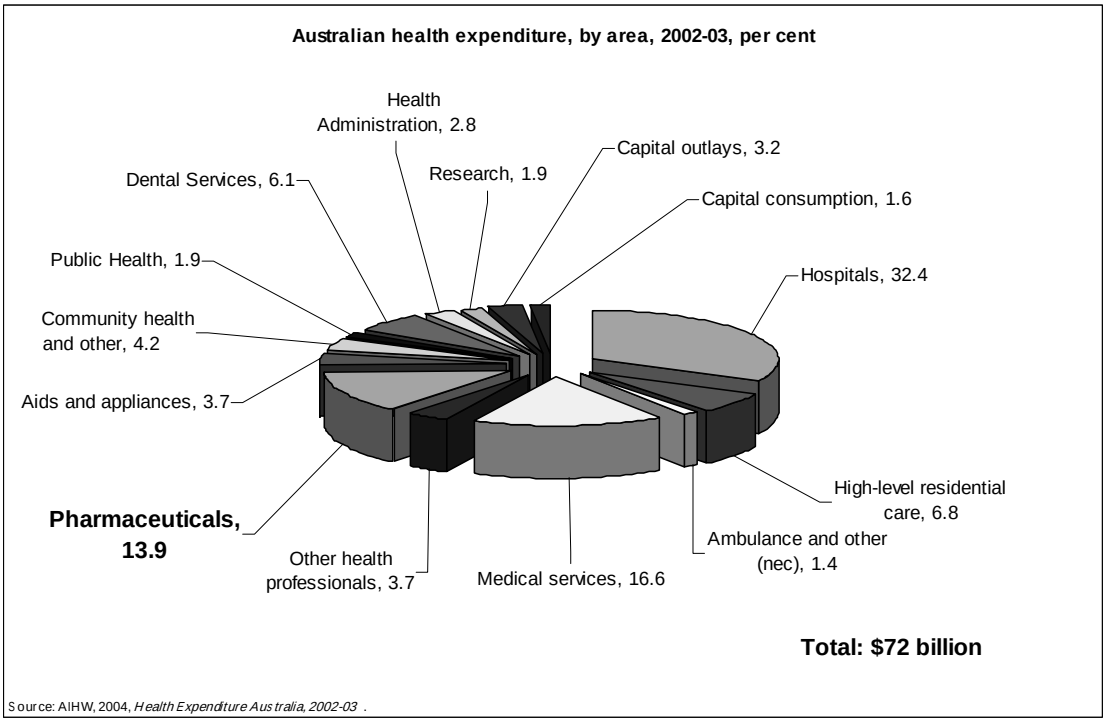
8.3 Australia's use of medicines

Australia currently spends $72 billion on health, or 9.5 per cent of GDP[70]. Of this $72 billion, the single largest component is hospitals which account for 32.4 per cent (Figure 12). This includes public, private and psychiatric hospitals. The next largest category of spending is medical services (16.6 per cent), representing doctors and specialists. Pharmaceuticals is third largest at 13.9 per cent, the bulk of which is accounted for by the PBS and is less than half of what Australia spends on hospitals. Other categories of Australian health expenditure include high-level residential care – including aged care facilities (6.8 per cent), dental

[70] AIHW 2004 *Health Expenditure Australia 2002-03*: Canberra.

services (6.1 per cent), community health (4.2 per cent) and aids and appliances (3.7 per cent).

Figure 12



Some concerns have been raised about the rate of growth in pharmaceuticals spending in Australia. Over the period 1991 – 2001, Australia has had a relatively high rate of growth in per capita spending on total pharmaceuticals (government and private) (Table 7). In fact, after adjusting for inflation, the growth in Australia's per capita spending on pharmaceuticals doubled. In the ten years to 2001, Australia's per capita expenditure on medicines grew by an average eight per cent each year in real terms, compared with 4.3 per cent in the previous ten years.

Table 7: Growth in OECD countries' per capita spending on pharmaceuticals[71], national currencies, 1995 GDP prices.

	Average annual growth rate (%)	
	1981-1991	1991-2001
Australia	**4.3**	**8.0**
Belgium	3.0	-
Canada	6.8	5.3
Czech Republic	-	7.0
Denmark	3.9	3.3
Finland	4.6	4.5
France	-	4.3
Greece	0.3	4.4
Hungary	-	3.3
Iceland	1.9	4.4
Ireland	3.0	6.1
Italy	-	2.3
Japan	-	1.7
Korea	-	6.8
Luxembourg	5.1	1.1
Netherlands	4.6	3.6
New Zealand	5.5	-
Norway	2.2	-
Portugal	8.0	-
Sweden	3.4	7.2
Switzerland	-	3.5
United Kingdom	4.2	-
United States	5.7	6.2

Source: OECD Health Data 2004.

However, the high rate of growth in the decade to 2001 should be put into perspective. Over the previous ten years (1981 – 1991) there were at least seven OECD countries that had higher annual growth rates than Australia. Three countries, Greece, Ireland and Sweden, also saw their average growth rate at least double in the period 1991 – 2001, compared to 1981 – 1991, much like Australia. Finally, all OECD countries for which there are data available have seen their spending on pharmaceuticals grow. Thus Australia is not unusual in having growth in pharmaceuticals spending, per se.

Compared to other OECD countries, Australia devotes a smaller share of its health spending to medicines. If the substitution of more labour-intensive medical treatments, such as hospital visits, for more cost-effective capital-intensive treatments like newer medicines represents a shift to greater efficiency, then Australia has some way to go to matching the performance of other industrialised countries. Australia's spending on pharmaceuticals is relatively low compared to other OECD countries (Figure 13, Figure 14).

[71] Total expenditure on pharmaceuticals and other medical non-durables comprises pharmaceuticals such as medicinal preparations, branded and generic medicines, drugs, patent medicines, serums and vaccines, vitamins and minerals and oral contraceptives. This classification is used throughout this section using OECD data and includes non-durables. Pharmaceuticals represent around 80 per cent of this expenditure, with non-durables accounting for 20 per cent.

Figure 13

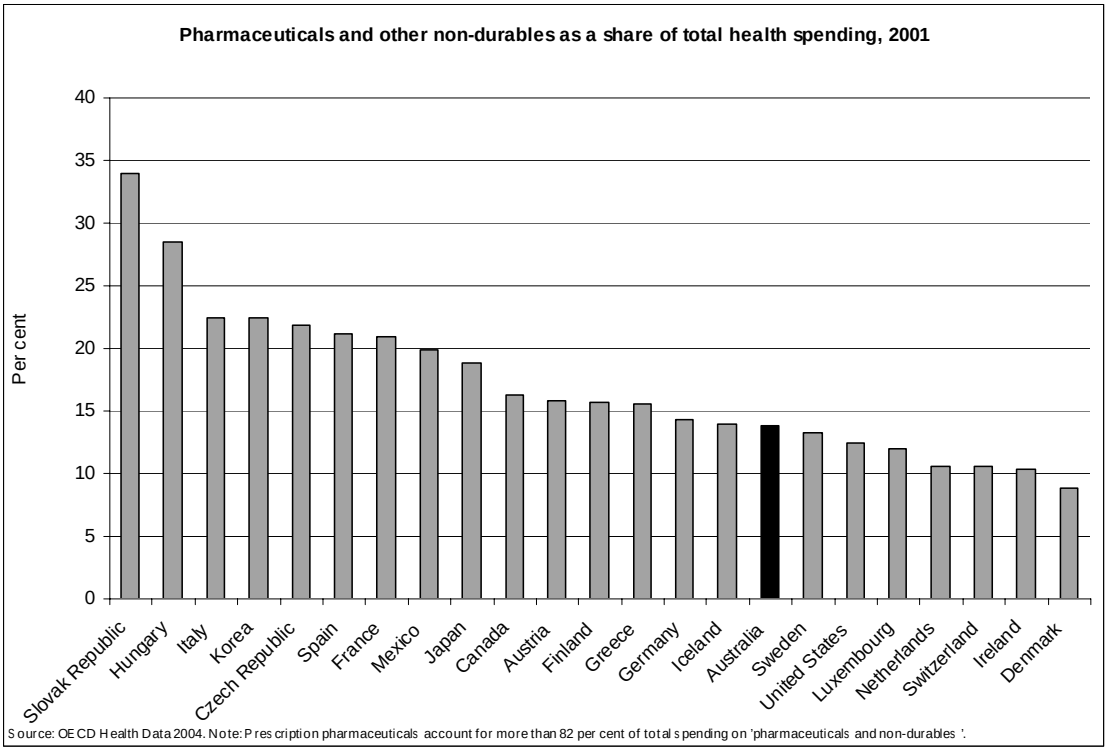
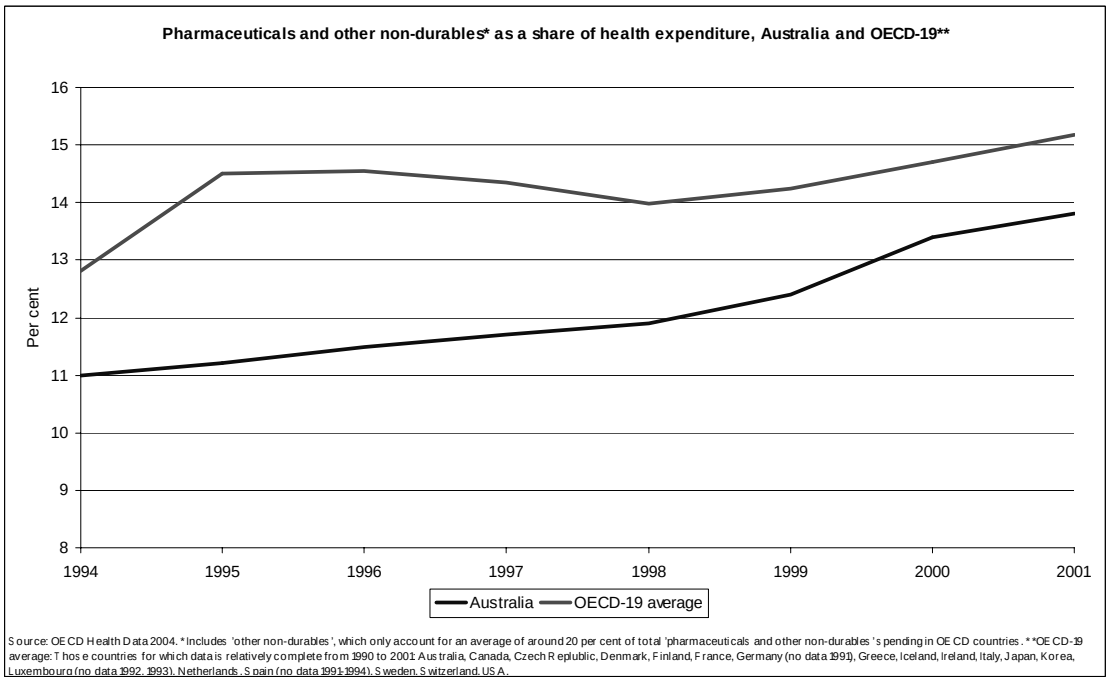


Figure 14



Interestingly, the role of medicines in countries' health spending varies. At one extreme, only 8.8 per cent of health expenditure in Denmark is spent on medicines in 2001[72]. On the other hand, the Slovak Republic spends more than one-third of its health budget on medicines. By comparison, Australia spent 13.8

[72] OECD Health Data 2004.

per cent of its health expenditure on medicines in 2001. While this is more than several industrialised countries, including the US, it is well below a range of countries that spend in excess of 15 to 20 per cent of their health budget on pharmaceuticals.

While some might view the growth in spending on medicines as a concern, the fact is that Australia spends relatively less on medicines than many other OECD countries. Moreover, if indeed the switch to using medicines instead of hospitals delivers overall savings in health care expenditure, as some of the literature on health outcomes suggests, Australia's current level of spending on medicines could perhaps be a concern.

Added to this is the fact that in Australia, pharmaceuticals are rigorously evaluated for cost effectiveness before being listed on the PBS. By and large, prescription medicines available have demonstrated cost-effectiveness through the PBS listing process, administered by the PBAC. The same cannot be said for most other treatments in Australia's health system, although applications for subsidy under the Medical Benefits Scheme (MBS) now require an economic evaluation.

The key point is that just because the cost of medicines, and the PBS, is increasing, albeit at a faster rate than other components of the health system[73], this should not necessarily be a cause for concern. More spending on medicines in Australia has the potential to provide net savings in other more labour-intensive parts of the health system, particularly hospitals. If more costly treatments are being replaced by newer, more effective innovative medicines, the overall impact on the health budget is actually a good thing. *"High-price new medicines may be the cheapest weapon we have in our struggle against rising overall medical expenses"*[74]*.*

8.4 Case studies of how medicines can reduce other costs in the health system

8.4.1 Mental illness

The development of a new generation of antipsychotic medications (so-called atypical antipsychotics) has significantly changed the management of psychotic disorders (schizophrenia and acute mania). Approximately 60,000 people in Australia suffer from schizophrenia and approximately 100,000 people suffer from bipolar disorder. Many of those with bipolar disorder will suffer from episodes of acute mania from time to time.

For many years, people suffering these disorders had the added burden of a range of acute and chronic toxicities associated with older medications such as haloperidol. In addition, these older medications were also limited in terms of effectiveness. While they dealt with the 'positive' symptoms of schizophrenia (hallucinations, delusions, aggression) they had little impact on the "negative symptoms (depression, loss of motivation and energy).

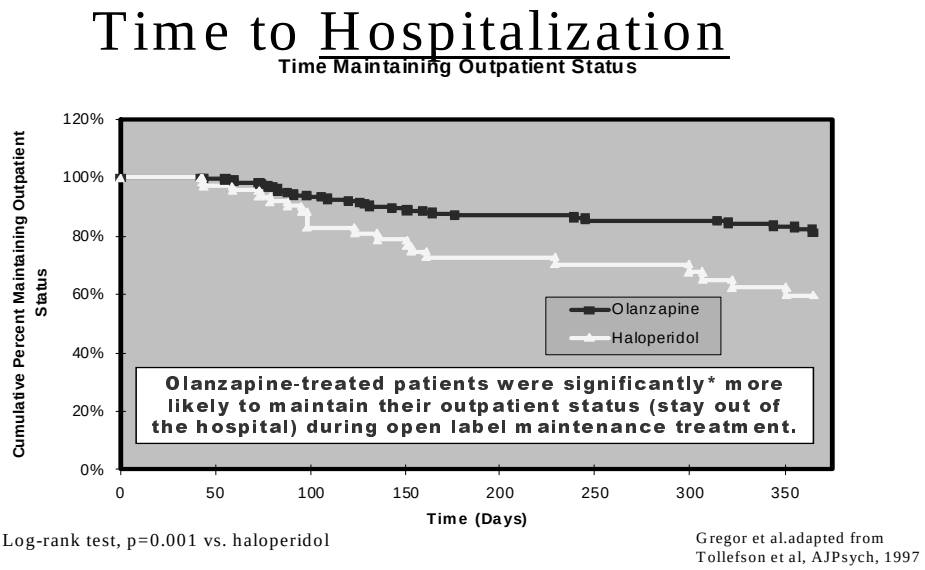
[73] PC 2004 *Economic Implications of an Ageing Australia: Draft Research Report*, November: Canberra, p. 6.6.
[74] Kleinke, J. 2001 "The Price of Progress: Prescription Drugs in the Health Care Market", *Health Affairs*, 20(5), Sept-Oct

The outcomes of therapy with these newer 'atypical' medications include better control of both positive and negative symptoms, better long term compliance, fewer acute relapses, less hospital admissions and shorter stays when hospital admission is required. An important factor in these outcomes is that the newer medications increase patient insight, cognition and engagement in therapy, enabling other non-pharmacological interventions to be more effective.

The evidence that expenditure on atypical medications offsets other health system expenditures comes from multiple sources: randomised controlled trials, Australian observational studies and international multi-country studies.

Figure 15 compares time to re-admission when treated with older antipsychotic (in this case, haloperidol) or newer 'atypical' antipsychotic (in this case, olanzapine).

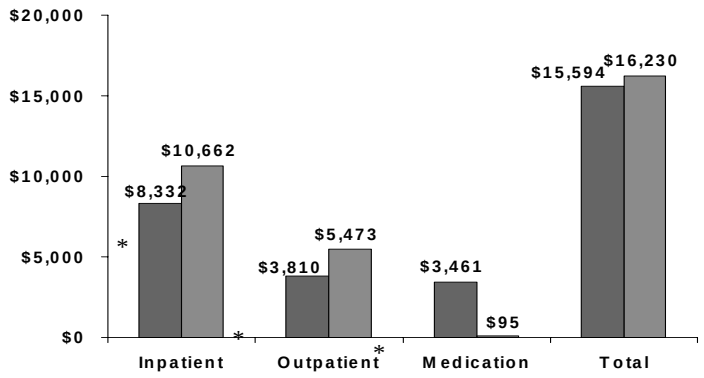
Figure 15



In Figure 16, in this US example using costing data from a randomized controlled trial, the higher medication costs when using the atypical antipsychotic (olanzapine) compared to the low cost older medication (haloperidol) was more than offset by the lower in-patient and outpatient costs.

Figure 16

Maintenance phase medical costs
US$ Per-patient costs over 46 weeks

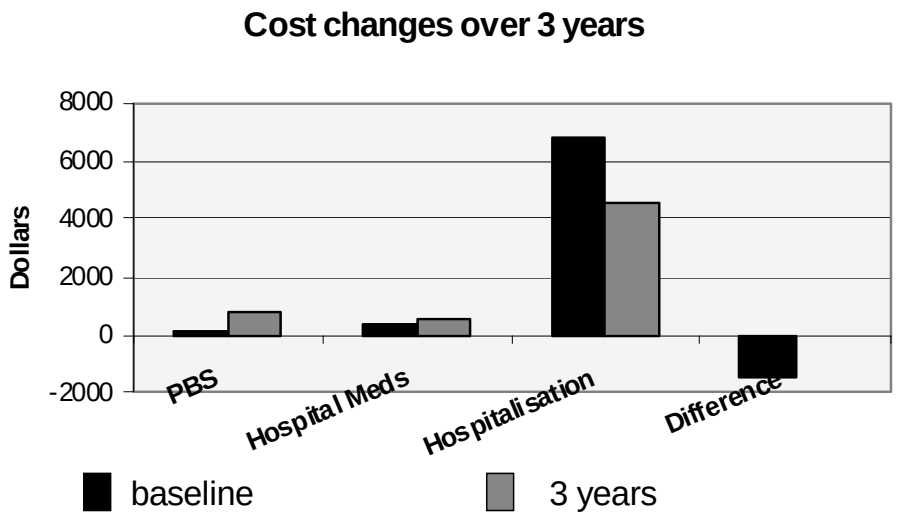


*F-test from regression model, $p < .05$ vs. haloperidol
Hamilton SH, Revicki DA, Edgell ET, et al. Clinical and economic outcomes of olanzapine compared with haloperidol for schizophrenia - Results from a randomised clinical trial. Pharmacoeconomics 1999;15:469-480.

Figure 17 shows an Australian example, where the total cost of treatment per patient declined by $2800 per year over the three years of an observational study. At the same time the use of newer "atypical" antipsychotics increased, leading to the increase in medication costs. In spite of this, the total cost of treatment declined, due to the reduction on hospitalization costs.

Figure 17

The cost per patient declined by $1400/6 month period ($2800/year)



M-TAG. Schizophrenia Care and Assessment Program - 3 year report. February 2004

8.4.2 Diabetes

Diabetes is a chronic disease characterised by high blood glucose levels. The two major forms of diabetes are Type 1 diabetes or insulin-dependent diabetes mellitus (IDDM) and Type 2 diabetes or non-insulin-dependent diabetes mellitus (NIDDM).

Type 1 diabetes is an auto-immune disease which is usually diagnosed in childhood or adolescence (but can occur in adults up to the age of 40). This form of diabetes requires insulin replacement. Its prevalence is much lower than type 2 diabetes but complications occur earlier and may be more severe.

Type 2 diabetes is the predominant form of diabetes in Australia. World-wide, it is one of the most common chronic disease among people aged 40 years and older and is one of the leading causes of death in Australia. The most recent data from the Australian Diabetes, Obesity and Lifestyle Study (AustDiab) suggests that Type 2 diabetes affects over 7 per cent of Australian adults,[75] however, only half of these individuals are diagnosed. This equates to more than 700,000 adult Australians, a figure which has doubled since the early 1980s and has been estimated to increase by more than 10 per cent in the last 5 years.[76]

The aim of therapy in both Type 1 and 2 diabetes is to improve control of blood glucose levels. Prolonged elevated blood glucose levels lead to vascular complications, both at a micro vascular level (causing blindness, kidney failure and nerve damage potentially causing amputations) and at a macro vascular level (causing cardiovascular disease and stroke).

Extensive research in the past 10 years has clearly documented the benefits of tight control of blood glucose levels (i.e. closer to the normal range). This can be accomplished by a variety of strategies depending on whether it is Type 1 or type 2 diabetes. These strategies involve optimal use of medications to control blood glucose levels. In Type 1 diabetes this involves administration of insulin. While insulin per se has been available commercially since 1922, a range of more complex insulin products have been developed in recent years enabling more flexible and convenient and increasingly effective insulin regimens to be prescribed.

In the case of Type 2 diabetes, treatment begins with improved diet and exercise and may progress as the diabetes develops to include a range of oral medications to reduce elevated blood glucose. A range of medications is available including relatively newly developed agents such as the glitazones that act by a different mechanism to the older agents.

The Diabetes Control and Complications Trial provided evidence that expenditure on diabetes medications offsets other health system expenditures. This very large US study compared the costs and benefits of applying intensive therapy to

[75] De Courten M. "Diabetes prevalence in Australia". *Presentation at the ADS and ADEA Annual Scientific Meeting*, August, Cairns, 2000. Abstract SY2.02

[76] Commonwealth Department of Health and Aged Care, Australian Institute of Health and Welfare (1999). *National Health Priority Areas Report: Diabetes Mellitus*, 1998. AIHW cat No. PHE 10. HEALTH and AIHW, Canberra.

those with Type 1 diabetes to maximize blood glucose control. As the slides below show, intensive therapy delays and slows the onset and progression of diabetes complications. This results in significant cost offsets (as well as improved health outcomes) particularly in the areas of reduced occurrence of retinopathy (diabetic eye disease) microalbuminaria (protein in the urine signifying the development of kidney disease), clinical nephropathy (the development of kidney disease) and clinical nephropathy (significant nerve damage potentially leading to pain and mobility issues and amputation).

Economics of preventing long term complications
Diabetes control and complications trial (Type 1): intensive therapy reduces complications

- Delays onset and slows progression of early complications of diabetes;
- Reduced the occurrence of:
  - Severe retinopathy by 47 per cent;
  - Microalbuminuria by 39 per cent;
  - Clinical nephropathy by 54 per cent;
  - Clinical neuropathy by 60 per cent.
- But at what cost and is it good value?

Cost of intensive therapy

- Resource use assessed in 29 centres participating in the DCCT;
- Therapy cost per patient:
  - Multiple daily injections and increased self monitoring: US$4000pa;
  - Continuous sub-cutaneous infusion (pump) US$5800pa;
  - Conventional therapy: US$1700pa.[77]

Determining value

- Modelled evaluation predicts benefits in terms of survival:
  - Conventional: average survival 56.5 years;
  - Intensive: average survival 61.6 years;
  - Gain of 5.1 years;
  - Cohort of 122,000 = 61,000 added years of life; and
  - Cost per life gained: US$28,661.[78]

As with Type I diabetes, improved blood glucose control through intensive therapy provides a number of cost-offsets by reducing the occurrence and severity of the long term complications. This was demonstrated in the large Prospective Diabetes Study conducted in the UK (UKPDS).

What about Type 2 diabetes?

- UKPDS intensive therapy increased costs by £478 per patient compared with usual practice;
- Cost of complications reduced by £957; and
- Cost per event-free year gained was £1167 (costs and benefits discounted by 6 per cent).[79]

[77] Source: DCCT Research Group, Diabetes Care 1995
[78] Source: DCCT Research Group, JAMA, 1996

8.4.3 Cardiovascular disease

A significant proportion of expenditure on the PBS is associated with the treatment and prevention of cardiovascular disease. Heart attack and stroke are the clinical endpoints of untreated hypertension and ischaemic heart disease. Management of blood lipid concentrations and blood pressure are routine tasks in general practice, with the result that medications for managing elevated cholesterol and elevated blood pressure are among the most frequently prescribed and highest total cost items on the PBS.

Conversely, the clinical management of the endpoints of acute ischaemic events (heart attack, coronary artery bypass surgery, coronary artery angioplasty and stroke) are expensive events within the Australian health system. As the technology available in these areas has increased, so has the cost per treated case.

There have been numerous studies of cost effectiveness of cholesterol-lowering medications. The PBS listing for these medications focuses on eligibility criteria designed to optimise their cost effectiveness. Ongoing research and evaluation is aimed at interpreting recent large clinical trials that suggest an even broader utilisation of these medications warrants consideration. All of these economic evaluations support the finding that the cost of treatment with 'statin' medications is substantially offset by reductions in more costly events and procedures related to cardiovascular ischaemic events. For example, an Australian group including the National Heart Foundation and the NH&MRC Clinical Trials Centre conducted an economic evaluation based on data from the 9000 patient LIPID trial.

This trial was conducted in 85 centres in Australia and New Zealand. After a mean follow up of 6 years, therapy with pravastatin (a widely prescribed cholesterol-lowering medication) was associated with:

- A mean reduction in all-cause mortality of 22 per cent;
- A reduction in hospital admissions for coronary heart disease and coronary revascularization of 20 per cent;
- A cost of medication with pravastatin of $4913 per patient;
- A reduction in hospital costs by $1385 per patient and of other long term medical costs by $360 per patient; and
- A cost per life year gained of $10,938, considered highly cost effective.

[79] Source: Gray A et al, BMJ, 2000

Table 8: Reasons for hospitalisation LIPID trial

Reasons for hospitalisation	Admissions		Length of stay		Cost per admission	
	Placebo	Pravastatin	Placebo	Pravastatin	Placebo	Pravastatin
Coronary artery bypass surgery	498	405	14.4	12.2	12165	11209
Unstable angina	1463	1258	4.6	4.3	2471	2452
Myocardial infarction	450	338	8.6	8.6	4873	4941
Angioplasty	331	240	5.9	5.4	4910	4795
Stroke	140	113	18.6	16.1	7273	6608
Other circulatory disorder	2916	2617	5.0	4.5	3329	3130

The overall cost-offset was $1667 per patient compared with the cost of pravastatin at $4913 per patient. Thus, the cost-offsets were approximately one third of the total cost of treatment with pravastatin.

8.5 Conclusion

While cost-offsets are taken into account in cost effectiveness evaluations reviewed by the PBAC, the fact remains that the silo approach to health budgeting means these offsets are often not communicated more broadly and no attempt is made to realise these offsets as savings. Some argue that hospital waiting lists mean that no savings can be realised by shorter stays or fewer admissions because, it is claimed, there will always be another person in the queue to occupy that hospital bed. However, the overall increased efficiency benefit to the health system as a result of such cost-offsets from medicines needs to be appreciated at the level of central agency in considerations of PBS expenditure.

Even within the PBAC process, cost offsets other than substitution of the new medicine for other PBS medications are not taken into account. While these cost offsets in other parts of the health system can be included in the cost effectiveness analysis in PBAC submissions, they are not considered when estimating the cost to the Commonwealth of listing the new medicine.

Assessing the impact of increased spending on medicines on health outcomes, productivity, workforce participation, health spending and economic growth is difficult. However, the evidence suggests that there are overall economic benefits from greater spending on medicines.

It is crucial that Government policies and processes take the potential cost savings of new, innovative medicines into account. For example, the Government's deliberations on an ageing Australia have not given sufficient recognition to the potential benefits to the health budget that spending on new medicines can offer. Medicines should not be seen simply as a financial burden on the budget that needs to be curtailed. Rather, the evidence suggests that in many cases greater spending on medicines leads to greater offsetting savings in other parts of the health system.

To ignore this trend may lead to less than optimal spending on medicines which in turn may make the health system less efficient. Australia may miss out on the broader trend of a more capital-intensive, innovative and efficient health system. This is in addition to the potential health, social and economic benefits of medicines discussed elsewhere in this submission.

9. IMPACT ON SOCIAL AND ECONOMIC OUTCOMES

In assessing how advances in medical technology influence healthcare expenditure, it is very important to capture all of the benefits arising out of that technology. In addition to savings in other parts of the health system, evaluations of new health technologies, such as innovative medicines, also need to consider the wider social and economic outcomes that arise from the use of newer medical technologies. If not, then beneficial new technologies may be incorrectly judged to be too costly or may not be sufficiently utilised. New medicines that improve the quality of life for patients or enable people to rejoin the workforce or allow people to participate in the community again need to be recognised for the benefits they bring. Such outcomes need to be better recognised in Australia at both a process and a policy level.

There is increasing evidence that innovative pharmacological interventions deliver benefits beyond managing symptoms, cure of acute episodes of illness or secondary prevention of other clinical syndromes or events. The examination of the broader consequences of interventions (not confined to medicines) is termed outcomes research. Outcomes may be defined as clinical, humanistic or economic.

Clinical outcomes include changes in frequency and severity of symptoms e.g. pain, or changes in symptom scores on recognised scales, such as the Positive and Negative Symptom Score used in schizophrenia research.

Humanistic outcomes include changes in health-related Quality of Life, or changes in functional activity, such as when expressed as Activities of Daily Living.

Economic outcomes include the costs associated with delivering the intervention and cost-offsets. Using the example of chemotherapy treatment, direct costs may include the cost of the medicine itself, the cost of outpatient administration and other medications required. Indirect costs refer to the cost of lost productivity as a result of premature death, inability to attend work (absenteeism) or reduced productivity on the job.

Cost-offsets describe the costs of other interventions or activities related to the treatment that are no longer required. This might include – using the chemotherapy treatment example – direct health cost-offsets such as the costs of surgery, hospital admissions and doctor visits. Non-health direct costs may also be relevant, for example cost of transportation to the clinic or childcare costs necessitated by the treatment.

9.1 Evidence for impact on humanistic outcomes

An increasing number of clinical trials of new medications now include measures of health-related Quality of Life (QOL). This impact may be measured using a range of general health and social research instruments (questionnaires) and/or disease specific instruments.

For example, a trial of a new medicine for the treatment of diabetes may include a general health-related quality of life measure such as the SF-36. This internationally recognised questionnaire is widely used to detect change in a range of social and functional abilities. Other similar measures include the EuroQol and the locally developed AQOL (Australian Quality of Life Measure). Such instruments are useful in determining change from baseline over the period of the study. These instruments have been rigorously validated including, in some cases, in the Australian population.

However, changes in a QOL measure alone are difficult to value, for example, what would be the economic value of a 10 per cent improvement on a QOL measure for diabetes? For this reason, it is important to quantify QOL effects using a health state utility measure.

The goal of health state valuation is to measure a patient's overall state of health during a trial, in order to incorporate that valuation into the overall economic evaluation via a cost-utility analysis. The utility measure may be obtained using a visual analogue scale, where the patient rates his/her health state on a graph or scale. Multi-attribute utility indices (MAUI) are another means of collecting this information using a series of weighted questions specifically constructed to enable calculation of an overall health state value. Examples of MAUIs include the ED-5D or the HUI (health utility index). It is also possible to derive utility values from certain QOL questionnaires such as the SF-36, using a specially constructed algorithm.

Utility values, traditionally, record full health as 1 and death as 0. Values between these points reflect the individual's health state: a value of 0.9 may reflect mild persistent pain and discomfort but with little functional impact, whereas a value of 0.6 may reflect more severe pain, other symptoms such as shortness of breath, and significant functional limitations.

The importance of the utility value is that it enables a 'quality adjustment' to be made to gains in length of life, potentially allowing comparison across a range of interventions. To illustrate with a simplistic example, a clinical trial may demonstrate that a new cancer treatment, X, prolongs life by 2 years. The cost of the treatment in that trial (including all medical costs) is $1,000 per patient. The value of that new treatment, therefore, could be regarded as $500 per life year gained.

If another new treatment, Y, prolonged survival by 6 months and cost $10,000 per patient, the value of that treatment would be $20,000 per life year gained. However, neither valuation takes into account the patient's quality of life during the period of treatment and extended survival.

Incorporating a utility measurement into the trials may demonstrate that, at the start of the trial, the patient's health state was recorded as 0.5, a low value reflecting their severe ill health. At the completion of the trial period the patient's health state may have improved to the level of 0.75, reflecting significant improvement in their quality of life.

This can be used to recalculate the value of treatment X which becomes $2000 per Quality Adjusted Life Year gained, while the value of treatment Y becomes $80,000 per Quality Adjusted Life Year gained.

While the PBAC provides no guidance on what is an acceptable cost effectiveness ratio, it is widely believed within the industry that the PBAC tends to recommend PBS subsidies for treatments with a cost per QALY of less than $40-50,000. Innovative products with higher cost per QALY figures have been recommended for subsidy (e.g. up to $70-80,000) but these are likely to be rare.

PBAC guidelines for submissions enable sponsor companies to include data on QOL measures and increasingly this is done. Where the QOL measure is included as part of a randomised controlled and blinded study, PBAC places considerable importance on the results. In these cases, QOL outcomes may be presented along with traditional efficacy and safety outcomes (i.e. how many patients in both arms of the trial reported changes in clinical outcomes such as symptoms or rating scales, how many reported adverse events).

However, when QOL is measured outside of a blinded trial context, industry's experience is that the results are often rejected by PBAC. The PBAC view is that, in these circumstances, QOL becomes a subjective measure easily influenced by the patient's awareness of which treatment they are receiving. While this may make the blinded collection of such data ideal, there are treatment and trial situations that prevent this ideal approach.

For example, the PBAC has rejected applications to reimburse Human Growth Hormone for use in adult patients with growth hormone deficiency. The main clinical problems experienced by these patients include fatigue, weight gain, general lethargy, depression and other minor symptoms associated with hormone deficiencies. The best holistic measure of these symptoms is the patient's QOL score which is usually below that of the general population.

However, evidence of improvement in the QOL score associated with human growth hormone therapy in adults, to date, has only been available from open label trials (i.e. trials where the patient and the investigating physician are aware of the treatment being received). In this example, the issue is that human growth hormone must be administered by daily subcutaneous injection and must be conducted for at least 6 months or more to demonstrate the effects. In this situation it would be unethical to subject patients to prolonged daily injections of a placebo and, in any event, such a trial design would not receive ethics committee approval.

It is important that gains in health-related quality of life are valued in their own right. As described above, a cost-utility analysis enables the decision maker to see the value of the new technology expressed in a format that provides the cost per Quality Adjusted Life Year (QALY). An increasing number of cost-utility submissions are presented to PBAC.

However, with a range of options available for a sponsor to use, it is impossible to agree on the single best utility measurement approach. As many trials of innovative medicines are designed primarily for regulatory approval rather than payer decision-making, the design of the trial may not be optimal for the collection of utility measures. For example, the trial:

- May be shorter than the time required to demonstrate the improvement in functional ability and QOL; and
- The medicine may be apply to a child and adolescent population, where little work has been done in establishing appropriate measures or standardising and validating existing ones.

All of these issues create uncertainty for the PBAC. They should not, however, constitute a reason for outright rejection of the submission. Instead, more agreement is needed on approaches and options along with recognition that, for some populations, it may be necessary to accept less than 'gold standard' data to reach a decision. The increased dialogue between the PBAC and industry referred to elsewhere in the submission may assist in dealing with these issues.

9.2 Evidence for impact on economic outcomes

The Government's Intergenerational Report acknowledged the need for a healthy ageing workforce as a key element to ensuring participation and productivity.

Implicit in any discussion regarding health care is the assumption that declines in health status can and do impact on workforce participation and productivity. Most modern studies of disease burden or cost-of-illness include indirect (or productivity) costs.

For example, the National Asthma Campaign used research from the Boston Consulting Group to estimate that the total cost burden of asthma to the Australian community in 1991 was between $585m and $720m, with around $320m in direct medical costs and around $260m to $400m in indirect costs from lost productivity. The estimate of $260m in indirect costs was attributed to direct absenteeism ($111m), caregiver absenteeism ($88m), reduced effectiveness at work ($40m) and travelling time to consultations ($23m).

Similar examples of the indirect cost burden for other chronic diseases in Australia (for example Alzheimer's Disease, diabetes, depression and heart disease) augment the evidence of the potential negative impact of chronic disease on productivity. The converse argument is that investment in medicines to reduce disease burden may well deliver benefits in terms of improved productivity.

A report from Australian health economist Paul Gross provides additional insight into this area[80]:

"… new evidence has emerged on how illness affects individual worker productivity, particularly for disorders where we expect modern medicines, per se, to exert a

[80] Gross, P. 2003 *The economic value of innovation: measuring the linkages of pharmaceutical research, use of innovative drugs and productivity gains*, Health Economics Monograph No. 80, Health Group Strategies, March.

significant impact. In theory, there are at least two levels of impact, one through illness and the other through risk factors that affect the worker's productivity.

Illness and productivity: US data [a] from employment surveys in the late 1990's suggest the following impact of specific diseases, mostly chronic diseases, on the worker productivity index (defined in this US study as the percentage of time that a worker is working at full potential):

Table 9

	Worker productivity index	*All employees surveyed Average hours lost per week due to*			
		Absence	*STD1*	*Productivity*	*Total*
All employees surveyed	*89%*	*0.33*	*0.28*	*3.82*	*4.43*
Digestive disease	*60%*	*0.58*	*5.66*	*9.72*	*15.96*
Mental health disorders	*67%*	*0.75*	*8.72*	*3.72*	*13.19*
Respiratory disease	*77%*	*0.75*	*2.65*	*5.85*	*9.25*
Injury	*79%*	*0.48*	*1.90*	*6.05*	*8.43*
Musculoskeletal conditions	*79%*	*0.74*	*6.12*	*1.38*	*8.24*
Cancer	*84%*	*0.30*	*5.54*	*0.74*	*6.58*

NOTE: STD= short term disability

This table suggests that the average US employee in this sample lost 4.43 hours per week, most of it in productive hours lost. Thus illness per se causes at least 12 per cent of available work hours to be lost.

While injury leads to the highest absence from work, mental disorders, musculoskeletal disorders have the highest losses in short term disability, digestive diseases have the highest losses in productivity, and in total hours lost, digestive disorders and mental disorders are ranked 1 and 2 and had the lowest worker productivity indices [b]. With the exception of injury, these are all chronic conditions that respond well to modern drugs.

While this conclusion is worthy of emphasis, we can also see that a number of other risk factors exert a toll on the workforce (mental disorders, high blood pressure, raised cholesterol and obesity), and many of these risk factors respond well to modern drugs."

a. WN Burton, DJ Conti. Business & Health, 1999, 34
b. Most of these conditions are responsive to modern drugs

US researchers Burton, Morrison and Werthheimer recently published a literature review of the validity of evidence about the economic outcomes of pharmaceutical interventions[81]. They reviewed articles published between 1990 and 2002 that were controlled prospective or retrospective studies and included a measure of productivity as an endpoint. Pharmacoeconomic modelling studies were excluded. Burton et al expressed productivity loss in terms of absenteeism and 'presenteeism' (at work or school but with diminished capacity due to symptoms).

Burton et al found that *"the evidence is very good for about a dozen medicine classes that pharmaceuticals reduce productivity losses caused by respiratory*

[81] Burton, W.; Morrison, A. & Wertheimer, A. 2003 "Pharmaceuticals and workers productivity loss: A critical review of the literature", *Journal of Occupational and Environmental Medicine*, 45(6), June, p. 610.

illnesses (i.e. asthma, allergic disorders, bronchitis, upper respiratory infections and influenza) diabetes, depression, dysmenorrhea and migraine." [82] In the majority of cases the evidence came from prospective randomized controlled trials.

In his paper *The Economic Value of Innovation: Measuring the Linkages of Pharmaceutical Research, Use of Innovative Drugs and Productivity Gains*, Paul Gross confirmed that higher levels of national health expenditures are associated with better health outcomes[83]. Moreover, better health outcomes obtained with modern innovative medicines lead to higher gross domestic product (GDP) by increasing both workforce participation and productivity.

A 2002 Access Economics report on schizophrenia[84] found that improved outcomes, dependant in part on access to newer antipsychotic medications, could reduce a projected $1 billion health burden associated with the illness. In 2001 the lost earnings from people unable to work due to schizophrenia was $488 million. Further investment on psychosocial and vocational rehabilitation is essential to maximise the effect of the investment in new medicines via the PBS. This exemplifies the need to regard PBS expenditure in a holistic fashion, not as an isolated silo of health expenditure. Further investment in basic biomedical research is also essential if we are to conquer the "brain and mind" disorders.

A more recent Access Economics report[85] notes that in Australia there were over 162,000 people with dementia in 2002. The prevalence of dementia is growing rapidly and will reach the 500,000 mark around 2040. Dementia cost over 117,000 years of healthy life in 2002 and will become the largest cause of disability burden in Australia by 2016. By mid-century, according to Access Economics, dementia costs may exceed 3% of GDP – unless we can find effective treatments.

In a 2002 National Bureau of Economic Research paper[86], Frank Lichtenberg confirmed that pharmaceutical technical progress has increased per capita output via its effect on employment rate and hours worked per employed person. Each successive vintage of innovative medicines has produced a progressive increase in per capita output. The research concluded that the use of new medicines reduces the rate of human capital depreciation.

A study in the United States by MEDTAP International[87] showed that spending on medicines has substantial health gains. For example, it showed that every dollar spent on medicines that lower a diabetic's cholesterol produces US$3 in health gains, each additional dollar spent on hormonal treatments for breast cancer results in at least US$27 of health gains, each dollar invested in beta-

[82] Ibid.
[83] Gross, P. 2003 *The Economic Value of Innovation: Measuring the Linkages of Pharmaceutical Research, Use of Innovative Drugs and Productivity Gains*, Institute of Health Economics and Technology Assessment: Dee Why.
[84] Access Economics 2002 *Schizophrenia Costs: an Analysis of the Burden of Schizophrenia and Related Suicide in Australia*: Canberra.
[85] Access Economics 2003 *The Dementia Epidemic: Economic Impact and Positive Solutions for Australia*: Canberra.
[86] Lichtenberg, F. 2002 *The Effect of Changes in Drug Utilization on Labor Supply and Per Capita Output,* Working Paper No. w9139, National Bureau of Economic Research, September: Cambridge, Mass.
[87] MEDTAP International 2004 *The Value of Investment in Health Care*: Seattle.

blockers to treat heart attacks produces US$38 in health gains, and every dollar spent on therapies to prevent strokes in high-risk patients has delivered health gains valued at US$2 to US$6.

In a 2004 article in *Finance and Development*, Professors David Bloom, David Canning and Dean Jamison found that better health has significant benefits for GDP growth[88]. They found that good health raises per capita incomes by improving labour productivity. Better health also leads to a greater incentive to save – lower mortality means saving for retirement becomes a major issue for people.

In an earlier 2000 article in *Science*, Bloom and Canning found that health influences GDP per capita in several ways[89]. Healthier populations tend to have higher labour productivity, suffer fewer lost work days from illness or need to care for family members that fall ill. People have stronger incentives to invest in their education because they enjoy the benefits over a longer time frame and tend to save for the longer term because of improved longevity. There is also a demographic dividend where lower infant and child mortality leads to a larger workforce.

The World Health Organisation has established that access to new knowledge-medicines and vaccines was substantially more important in achieving the dramatic decline in mortality rates throughout the twentieth century than income growth, improved educational levels and improvements in nutrition and sanitation.

Further academic studies have shown that the use of prescription medicines reduces absenteeism of chronically ill workers and increases their productivity by a value far greater than the cost of the medications. Other studies have shown that poor health has a substantial impact on a person's earnings, workforce participation and productivity.

9.3 Current Australian approach to indirect costs

The guidelines for submissions to the PBAC take a societal approach to economic evaluation. This implies that all costs and benefits can (and should) be included in an evaluation. The issue of how to regard indirect costs was the subject of a literature review commissioned by the Department of Health in 1997. However, the topic was never really resolved and, from an industry perspective, continues to be a subject for concern. Technically, the guidelines permit the inclusion of data on indirect costs, such as lost productivity due to premature death or absenteeism. However, appendices to the guidelines impose stringent and limiting conditions on the use of this data stating that:

"In general, changes in productive capacity as an outcome of therapy are not encouraged in submissions to the PBAC. While this may improve the quality of life for the patient and could be included, quite legitimately, in a quality of life

[88] Bloom, D.; Canning, D. & Jamison, D. 2004 "Health, Wealth and Welfare", *Finance and Development*, 41(1), pp. 10-15.
[89] Bloom, D. & Canning, D. 2000 "The Health and Wealth of Nations", *Science*, 287, 18 February, pp. 1207, 1209.

scale, it should not be assumed that there is an economic benefit to society through the patient's return to productive capacity.

The reasons for this are:
a. For short term absence, production will be made up on the return to work;
b. Employers usually have excess capacity in the labour force to cover absenteeism; and
c. For long-term absence, production will be made up by a replacement worker otherwise unemployed
In Australia, the economy is constrained by macro-economic factors rather than by the lack of healthy workers. Productivity estimates give the misleading impression that additional output in the economy will pay for the additional drug consumption. If consideration of such indirect benefits can be justified in the submission, the following standard economic practice should be adopted:
a. Present the results both with and without the indirect benefits and costs included
b. When assigning a monetary value to the estimate of potential working time gained or lost in time units, the underlying assumptions must be made explicit. For example, the claim that there has been recovery of production lost to illness is dependant on demonstrating that

- *i. The worker returns to work*
- *ii The worker is productive*
- *iii. The work lost is not made up elsewhere by others in the company or the same worker following return to work (NB if the worker is highly productive, the incentives to replace him/her are stronger); and*
- *iv No temporary replacement from outside has been employed (namely that there is full employment).*

The net effect is that the marginal increase in production due to return of healthy workers to the workplace is over estimated by simply multiplying the worker's time regained by the labour market value of the workers (usually estimated by their wages). It is not always likely to be zero either, but some proportion in between. The evaluation should estimate the true proportion based on firm evidence."[90]

Detailed evidence of this kind is generally not available from clinical trials. Moreover, the claim that the lack of healthy workers is not a significant constraint on Australia's economy does not acknowledge the concern about the impact of an ageing population on future economic prosperity. One of the concerns currently being debated is exactly whether an insufficient number of healthy workers in the future could constrain Australia's economic growth. As argued elsewhere, innovative medicines will be a key tool in ensuring that the Australian workforce remains active as the population ages.

[90] DoHA 1995 "Appendix I: Estimating the present value of costs and health outcomes", *Guidelines for the Pharmaceutical Industry on Preparation of Submissions to the Pharmaceutical Benefits Advisory Committee: including major submissions involving economic analyses*, http://www.health.gov.au/internet/wcms/publishing.nsf/Content/health-pbs-general-pubs-pharmpac-gusubpac.htm-copy3 (accessed 23/11/04).

Most double blind randomised controlled trials of pharmaceuticals are conducted on an international basis for regulatory approval purposes. They are usually designed with clinical endpoints in mind although increasingly, humanistic endpoints (such as QOL measures) are being included. Less frequently, but not uncommonly, phase III trials are also measuring resource utilisation (doctor visits, hospital admissions, concomitant medications etc). These data are then available to calculate direct health care costs, which can be utilised in cost-effectiveness or cost-minimisation analyses.

The challenge for industry in Australia arises when a sponsor wishes to demonstrate that the new product for a chronic illness improves productivity through decreased absenteeism. Such data is rarely captured in a phase III trial, in part because patients are often selected as a sample of convenience (i.e. hospital clinic patients) and/or the duration of the trial is too short to demonstrate this effect. Therefore data on productivity impact often comes from less rigorous studies designed to explore the risks and benefits of the product in the real world setting. While these may be randomised studies, they are unlikely to be blinded (or indeed 'double blinded'). Other research methods may include observational studies or surveys.

Because these studies fall short of the PBAC's preference for gold standards of evidence, sponsors are encouraged to submit cost-effectiveness analyses with and without the indirect costs included. Industry is concerned that this approach minimises the likelihood that indirect costs will be considered seriously. Moreover, the silo nature of health funding means the assessment of new medicines tends to focus predominantly on direct health costs to the PBS and much less, if any focus, on the impact on indirect costs.

The industry acknowledges that this is not an easy issue to resolve and that it requires a whole of government response. If the view is sustained that indirect costs are not appropriate within PBAC evaluations, then it may be useful for dialogue with the Departments of Finance and Treasury on how these indirect benefits could be quantified elsewhere in the reimbursement approval process. For example, when Cabinet considers new products proposed for the PBS (ie. those which are expected to incur costs of $10m and above), indirect economic benefits could be given greater consideration.

10. CONCLUSION: IMPLICATIONS FOR THE FUTURE

10.1 Guidelines review

The PBAC guidelines have been reviewed several times since their introduction in 1992. The latest review is underway and being conducted by the Economic Subcommittee of the PBAC. The plan for the review calls for a section-by-section process, with drafts of revised sections being posted on the web for comment by stakeholders. The scope appears broad and designed to include a number of topical issues.

Of note is the proposal to include several new appendices covering topics such as trial based utility valuation, scenario based evaluation and monetary valuation. Hopefully these will add guidance to areas that are often points of contention. In the later case, willingness-to-pay methodologies have not previously been included in the guidelines but have been used in some submissions to address specific valuation needs.

Modelling as a component of the economic evaluation is another complex area that is being revised. The PBAC and PBB have identified four main goals for models. In their view, models should:

- Contribute to describing the 'most plausible' and 'policy relevant' estimates;
- Identify characteristics and impact of the main drivers of the estimates and their role in any uncertainty;
- Be more explicit regarding the place for the proposed treatment and how it fits with alternative treatment options; and
- Examine other options via scenario testing.

The industry response to these is agreement with the first two and caution in regard to the latter two. In particular, industry sees the construction and population of models designed to test multiple alternative product positioning scenarios as extremely difficult. Models of one proposed scenario (as currently submitted) are complex enough and often challenged over sources of data and assumptions. The desire of PBAC to look at multiple scenarios is understandable. However, at the level of actual data and outputs, expanding these models to cope with multiple positioning scenarios appears likely to create further uncertainty rather than resolve it.

10.2 Quality Use of Medicines and Health Technology Assessment

Australia's National Medicines Policy includes the Quality Use of Medicines as one of its pillars. The QUM component is under the stewardship of the Ministerial advisory committee on Pharmaceutical Health and Rational Use of Medicines (PHARM). Unfortunately the term QUM has not been well understood by all stakeholders and PHARM is working to address this.

The Chairman of the PBAC has asked PHARM to consider how the principles of QUM could be incorporated into the PBAC process. This is a work in progress, with a draft discussion paper completed and inputs being provided by DoHA. The next step is likely to be a workshop of stakeholders to progress the general approach. One area that has been made clear is that any proposal in a PBAC

submission for a company-supported QUM program should not be seen as compensation for poor cost effectiveness. Rather, the concept does provide an opportunity to increase the certainty that clinical and cost effectiveness outcomes described in the submission will actually be achieved.

10.3 Positive outcomes in implementing recent changes to the PBS

10.3.1 The US-Australia Free Trade Agreement

The US-Australia FTA (FTA) facilitated a constructive dialogue about the listing process and reward for innovation. The industry welcomes initiatives under the FTA to increase transparency and accountability in PBAC decision making through:

- Increased opportunities for interaction between companies, the Department of Health and Ageing and the PBAC - this may include hearings before the PBAC's sub-committees as well as the PBAC itself; and
- The opportunity to seek an independent review of a PBAC decision.

The expected benefits of these initiatives include:

- Earlier identification and resolution of issues in company submissions before they are considered by the PBAC;
- Clarification of PBAC's needs and expectations of clinical data; and
- Increased clarity and communication of information about reasons for acceptance or rejection of submissions.

The FTA did not however address the policy framework for the PBS and therefore presents no solutions to industry's concerns with the PBAC's models for economic evaluation and cost effectiveness analysis. The industry applauds continued initiatives to work collaboratively with government to find mutually agreeable policy solutions, but this collaboration needs to be more regular and involved.

Initiatives under the FTA will deliver incremental improvements over time. Negotiations about their implementation are progressing well, with all parties committed to a system that is both workable and sustainable.

10.3.2 The post PBAC (pPBAC) processes review

In August 2003, The Department of Health and Ageing and Medicines Australia (representing the pharmaceutical industry) commenced a joint review of the post PBAC processes. The review did not open up for debate or negotiation the policy framework for either the PBAC's models for evaluating medicines proposed for PBS listing or the PBPA in determining pricing.

Instead it provided the Department and Medicines Australia with an opportunity to work together to develop a more effective, efficient and transparent listing process within existing policy settings.

The pPBAC review focused on those steps of the listing process which take place after the PBAC has made a recommendation and publication of the listing in the Pharmaceutical Benefits Schedule (the 'yellow book').

The findings of the review were presented to the Minister in June 2004. Since then, the Department of Health and Ageing and Medicines Australia have continued to work collaboratively on implementing the findings of the review. This work continues.

Major benefits expected from the pPBAC review include:

- Reduced timeframes for listing approved PBS items;
- Opportunities for earlier and more frequent discussions to resolve 'hot spots' in pricing;
- Faster resolution of restrictions wording; and
- Introduction of a monthly cycle for listing PBS approved items.

10.4 Need for reform of the process of health technology assessment

The pressures on the PBS are not new. Concerns about the growing total cost of pharmaceuticals, the impact of ageing, and the cost of developing and acquiring new technology are rarely viewed in terms of the impact on the total health system. In the assessment of technologies for pharmaceuticals, we see the most stringent assessment of value, and that market access is dependent on this assessment. Because the supply of pharmaceuticals is a global business, it also differs from some other health technologies and interventions.

Policy change is required to address the issues identified in this submission. Without change, the gap between new pharmaceuticals and Australians' potential access to those medicines will grow, because there will be more pressure on the ability of new products to demonstrate value. Uncertainty will always be a part of decision-making but it needs to be managed differently.

In addition to these continuing trends, the nature of pharmaceuticals will be impacted by new technological approaches, including the emerging challenge of demonstrating cost effectiveness for biopharmaceuticals and other new products. Another important consideration is the growing demand by consumers for preventive medicines that, for example, aim to delay disease onset (eg Alzheimer's) or delay and minimise longer term complications of chronic diseases.

It is important that the PBAC proactively considers how to manage the impact of issues such as the lack of appropriate comparators in these subset conditions, how existing cost effectiveness methodologies will deal with assessing long term health gains and, for industry, how companies will be able, in the face of continued pricing pressure, to recoup the significantly higher research and manufacturing costs of biopharmaceuticals.

Medicines Australia submits that the system and its processes would benefit from a solution-focused approach to listing of new medicines, given the common aim is to find mutually agreeable solutions to making appropriate medicines available to the consumers.

10.5 Conclusion

The development of new, innovative medicines, like advances in other areas of medical technology, open up new opportunities to treat disease, extend lives, enhance quality of life, improve economic productivity and provide cost savings. A significant part of the improvement in human health in Australia and worldwide over the years has been due to the development of new medicines. Like many other countries, Australia has the challenge of ensuring that its citizens have access to these new technological developments. One-dimensional concerns, both at a process and a policy level, about increased costs run the risk of ignoring the plethora of benefits for society from the availability of such new technologies.

This submission focuses on the application of the inquiry's terms of reference to the case of innovative pharmaceuticals and their role in advancing medical technology. Medicines represent a technology that is continuously evolving, subject to very high research and development costs and complex risk assessment processes.

Medicines Australia supports the Pharmaceutical Benefits Scheme as an important piece of Australia's health system and National Medicines Policy. The PBS depends on a very sophisticated and complex Health Technology Assessment process utilising economic evaluation as the means of making purchasing and valuing decisions.

Both the government and the pharmaceutical industry have made a large investment in HTA. Given the size of government expenditure on the PBS, this may be appropriate. However, Medicines Australia also believes there a number of areas that need to be considered with regard to Australia's approach to HTA and pharmaceuticals.

Australia has a complex system of evaluation, subsidy, pricing and reimbursement related to pharmaceuticals. This complexity and inter-related mechanisms are not well understood by policy makers, prescribers and consumers. The actual process as applied under the PBAC guidelines is extensive, complex and uses theoretically ideal standards that are often difficult to achieve in terms of global pharmaceutical research and development. Aspects of evaluation, determination of cost-effectiveness and handling of costs and benefits need increased flexibility if the continued gains in biomedical innovation are to be made accessible to Australian consumers.

As a specific example, the issue of eroding prices for comparator products, even while medicines are still under patent, makes the demonstration of 'appropriate cost effectiveness' increasingly difficult. This delays access to innovation and will continue to restrict access by Australian patients to medicines that may well be very appropriate for them in terms of delivering better health outcomes. Some form of price indexing for comparator products is critical if future innovation is going to get to Australian consumers.

The saving of other costs within the health system needs to be better recognised and communicated up and down the health system. Where an innovative pharmaceutical is shown to reduce hospital admissions, length of stay or use of other medicines and services, this needs be recognised as true improvement in economic efficiency terms.

On the other hand, significant improvements are being made to the operation of PBS processes. These are largely as a result of the US-Australia Free Trade Agreement and include improvements in independent review, increased interaction between PBAC and sponsors and increased transparency. Some improvements have also been made in specific pricing policies e.g. the operation of the complex Weighted Monthly Average Treatment Cost policy (WAMTC).

The overall pattern of growth in healthcare expenditure on pharmaceuticals needs to be understood and, in and of itself, may not need be a cause for concern. Demand for technology in terms of innovative pharmaceuticals is driven by a number of factors, not least being Australia's National Health Priority Areas and the evolving evidence base for these interventions.

The benefits of medicines, broadly defined, need to be recognised more generally in terms of appropriateness of the investment. Government policy across the board needs to recognise the benefits of the availability of the latest, innovative medicines in Australia. While the processes of assessing medicines need to consider the benefits of medicines, so too the broader policy debate about the role of innovative medicines in Australian society needs to recognise these benefits.

The research-based pharmaceutical industry in Australia is committed to developing and delivering innovative pharmaceuticals. Medicines Australia hopes the Productivity Commission will recognise the strengths of the HTA process applied to pharmaceuticals but also recognise areas where reform is needed.

Medicines Australia and its members continue to consider the policy options and mechanisms available and to work with Government in developing these further. We are very willing to engage in consultation with any stakeholder in order to improve equity of access for Australia consumers alongside appropriate recognition of innovation. It is through this process that the role of innovative medicines in enhancing life and society can be better understood.

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