

21 December 2004

Ms Helen Owens  
Commissioner – Medical Technology Study  
Productivity Commission  
LB2, Collins Street East  
Melbourne VIC 8003

Dear Ms Owens

GlaxoSmithKline (GSK) welcomes the opportunity to contribute to the Productivity Commission's study into the impact of advances in medical technology on healthcare expenditure in Australia. The study is both important and timely, in that it pre-empts the imminent arrival of significant technological thresholds in the pharmaceutical industry and broader health sciences as well as focusing on the current public and policy debate surrounding the ageing of the Australian population.

### **GSK Background**

GSK is a world leading, research-based pharmaceutical company dedicated to meeting the healthcare needs of people around the world and helping them do more, feel better and live longer. The company is a global leader in the research, development, manufacture and supply of prescription medicines, vaccines, over the counter medicines, oral care products and nutritional healthcare drinks.

At the forefront of the rapid progress medical science, GSK is committed to sustaining its current R&D intensity and investment. The company allocates approximately \$A6.7 billion (£2.8 billion) to R&D annually and has a significant product pipeline of new chemical entities and vaccines in clinical development. Within Australia, GSK invested over \$A36 million in R&D in 2003, making it one of the largest contributors to business investment in R&D.

With a longstanding commitment to the pharmaceutical industry, GSK offers substantial insight into its diversity and complexity. The company's perspectives are underpinned by an understanding of future directions in pharmaceutical technology and commitment to the long-term sustainability of the industry in Australia.

### **Medical Technology & Healthcare Expenditure**

There is widespread consensus, both in Australia and other developed countries, that the development and diffusion of medical technology has been one of the key drivers of increasing healthcare expenditure in recent decades<sup>1</sup>. Debate exists, however, regarding the magnitude of that contribution. While most new technologies impose direct costs on the healthcare system, they deliver major offsetting savings in other parts of the system, for example fewer and shorter hospital stays or reduced need for aged or disability care services. However such savings often become apparent only over very long periods of time. Moreover, with split responsibilities for funding and delivery of services, they can be hard to capture in their entirety or to allocate across the health care environment. As a result, "it is difficult to be clear about the impact of accelerating innovation in health care on long-term health costs"<sup>2</sup>. This is an area which would benefit from further research.

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1 Busse et al. Shaping the Societal Bill. Futures Vol.35 Iss.1 2003; Commonwealth Government, Budget Paper No. 5 – Intergenerational Report 2002-03;

Cutler, D.M. and McClellan, M. Is Technological Change in Medicine Worth It? Health Affairs. Vol.20 Iss.5 2001

2 Sheehan, P. Health Costs, Innovation and Ageing. Centre for Strategic Economic Studies. August 2002

## Medicines as Medical Technology

Medicines are a distinct and important subset of medical technology. Medicines save lives, relieve pain, cure and prevent disease. They help improve quality of life for patients and caregivers and enable employees to stay at work and remain economically productive.

Over the last century, vaccination has effectively eliminated many diseases which caused substantial disability and premature death, while medicines have changed the way other diseases are treated, reducing their burden on patients, health systems and the community.

Medicines are an effective and generally economical means of treating and curing disease, with research consistently demonstrating that expenditure on medicines is associated with greater offsetting savings in hospital and other health expenditures. For example:

- Each additional dollar spent on diabetes in the United States in the last 20 years has saved \$1.49 in other health care system costs, by reducing the length of hospital stays and the risk of complications developing such as stroke, heart failure or amputation<sup>3</sup>;
- In the ten years following the introduction of stomach-acid-blocking H2 antagonist drugs in Canada there was a reduction in surgeries to treat stomach ulcers from, 97,000 to fewer than 19,000. The cost of therapy using medication was about \$900 per person, whereas the cost per person for surgery was around \$28,000<sup>4</sup>; and
- Following the introduction of antiretroviral therapy for HIV patients in the US, researchers found a 43% decrease in hospital in-patient care, and although pharmaceutical expenditure increased by 33%, overall health care expenditure fell by 16%<sup>5</sup>.

However, medicines cannot be viewed solely through the prism of reducing other healthcare costs. New medicines frequently address unmet medical needs, or improve outcomes for patients where current treatments are associated with adverse side effects. For example:

- New and innovative medications for asthma have resulted in a 28% decline in mortality from the condition in Australia over a ten year period during the 1990s<sup>6</sup>,
- The introduction of community vaccinations for polio from the 1960s onwards has resulted in annual deaths in Australia falling from over 1,000 to zero; and
- Prescription medicines for Type II diabetes in the US resulted in savings for employers of \$1,475 per employee per year due substantially to reduced absenteeism<sup>7</sup>.

Capturing the full societal and individual benefits of new medicines is a complex task. In addition to the previously mentioned challenges of tracking monetary savings through the health system over time, high-quality cost effectiveness analysis needs to accurately measure and value more intangible health outcomes such as longevity, productivity and quality of life. Australian health authorities, as international leaders in the adoption and adaptation of cost effectiveness analysis techniques in the assessment of new medicines, have a very important role to play in developing the methodologies and data with which to achieve this objective.

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3 MEDTAP International, "The Value of Investment in Health Care: Better Care, Better Lives," (Bethesda, MD: MEDTAP, 2003).

4 Pharmaceutical Researchers and Manufacturers of American, "The Value of Medicines", Washington, 2001.

5 Bozzette S et al, "Expenditures For the Care of HIV-infected Patients In the Era of Highly Active Antiretroviral Therapy," NEJM 344 (2001).

6 Woolcock, A.J., et al, The Burden of Asthma in Australia, Medical Journal of Australia, 2001.

7 Rizzo J. et al, "Labor productivity effects of prescribed medicines for chronically ill workers," Health Economics, Vol 5.

## **Future Directions for Medical Technology in Australia**

Medical technology, particularly pharmaceuticals, is on the verge of major and fundamental advances. GSK alone has 148 new products in clinical development, including paradigm shifting vaccines like *Cervarix*<sup>™</sup> for the prevention of cervical cancer in women, and a range of new compounds which have significant potential to fight neurodegenerative and psychiatric diseases such as multiple sclerosis, Alzheimer's and schizophrenia.

Australia will inevitably be impacted by these new technologies. International markets are driving demand for innovation and increasingly free flows of information about emerging medicines will continue to drive calls for access by local physicians and patients.

Finding appropriate ways of assessing the cost and health effectiveness of these innovations may prove challenging and will require improved data from all sources, a more holistic means of considering all sources of funding within the healthcare system and greater transparency and agreement around the value of the benefits associated with increased life expectancy and quality of life.

## **Research by the Allen Consulting Group**

In preparing this response to the Productivity Commission, GSK has commissioned an independent report by the Allen Consulting Group. The Group has utilised their considerable expertise in the Australian health sector as well as extensive historical health data in undertaking this report, which is attached.

The report examines trends in pharmaceutical use and expenditure in Australia, highlighting the factors which have underpinned increases over the last decade. It highlights the need for a more holistic assessment of the impact of advances in pharmaceuticals, presenting a case study on medicines for the treatment of heart disease to illustrate the potential for new medicines to generate significant reductions in other health expenditure and increases in broader economic and social benefits. It concludes with a discussion of proposals based on risk-sharing between the Government and the pharmaceutical industry which may provide options for managing the funding of revolutionary new medicines in the context of significant uncertainty about both benefits and costs.

GSK would welcome the opportunity to further discuss this report or to answer any questions relating to the attached report. Please do not hesitate to contact me on 03 9721 6608 if we can be of further assistance.

Yours sincerely

Alex Gosman  
Director – Healthcare Environment

The Allen Consulting Group

## **Managing the benefits and costs of advances in pharmaceuticals**

Submission to Productivity Commission study on the impact of  
advances in medical technology

December 2004

Report to GlaxoSmithKline

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## Executive summary

### **The impact of pharmaceuticals: a broader view**

There has been a great deal of focus recently on the use of and expenditure on pharmaceuticals, with concerns raised about the capacity of the public sector to fund the projected growth in the longer term. This focus is understandable, as the data show increasing usage of pharmaceuticals with an associated significant increase in expenditure.

Furthermore, the factors underpinning the increase in usage and expenditure, including developments in technology, the ageing of the population and consumer preferences, are likely to continue and even strengthen. For example, developments in pharmacogenomics — the study of how people’s genes affect the way they respond to medicines — have the potential to lead to innovative therapies for the better prevention and treatment of diseases, but often at relatively high cost.

However, it is not simple to assess the appropriateness of the increasing expenditure on pharmaceuticals in Australia. Whether the expenditure is too little, too much or just right depends on the nation’s preferences, the opportunity costs of the expenditure, and in particular, the cost effectiveness of the expenditure. This requires a comprehensive understanding of the costs and the benefits of pharmaceuticals.

Discussions about the impact of advances in pharmaceuticals tend to take a fairly narrow approach to the assessment of their costs and benefits — indeed often to focus solely on the direct costs. Considering the expenditure on pharmaceuticals separately instead of as part of overall resource use in the healthcare system has been criticised as reflecting a ‘silo mentality’. Advances in pharmaceuticals are generally associated with a change in the way healthcare is delivered, with pharmaceuticals often substituting for other medical interventions. From this perspective, expenditure on pharmaceuticals may often be a substitute for, and hence reduce, other healthcare expenditure, and it is the *net* effect on total healthcare costs that needs to be considered, in relation to the benefits.

Similarly, the benefits of pharmaceuticals are often considered only in terms of direct effects on health outcomes. Important as those are for people’s wellbeing, a broader appreciation of the benefits — including, for example, the economic benefits of reducing productivity losses due to poor health — allows a more comprehensive view. Employers benefit from reduced absenteeism and overall improvements in employees’ health; taxpayers also benefit through reduced government outlays on welfare and a higher tax base associated with a more productive workforce. In addition to these societal benefits, the positive impact of pharmaceuticals on individuals — in terms of the improved quality of life that follows from better health — must also be acknowledged in an assessment of the benefits of pharmaceutical innovations.

This report presents a case study modelling the improved take-up of pharmaceuticals for the treatment and prevention of heart disease, which illustrates this broader view. It shows a significant reduction in expenditure on hospital care, pointing as well to a likely increase in workforce participation for mature age people at risk of heart disease.

### **Risks and uncertainty**

Nevertheless, it is fair to say there is significant uncertainty associated with both the benefits and related costs of advances in pharmaceuticals, particularly from the perspective of this broader view. This is the case for both the government and the pharmaceutical companies.

From pharmaceutical companies' perspective, innovation is complex and comes at a high cost to the developers. Developing, testing, and gaining approval for a new medication is very costly, time consuming and risky. To preserve incentives for innovation given the risks, it is necessary to allow innovators not only to recover their costs but also to make adequate profits where discoveries lead to effective treatments. This is offered through the patent system and pricing policies which balance adequate returns on investment with affordable access to essential medicines. The situation for pharmaceutical companies is analogous to that of companies prospecting for oil or other minerals — they must recover enough on the wells (or mines) that do go into production to cover their total discovery costs and associated risks.

From the perspective of the Government, which has responsibility for approving the listing and pricing of pharmaceuticals and is the main source of funding, risk management is a crucial element of its pricing responsibilities. If the Government *spends too much* on pharmaceuticals, it risks adverse events following from too little expenditure on alternative investments in health or other goods and services. It also risks over-utilisation, which is not only inefficient but could also lead to poorer health outcomes in some cases. On the other hand, if the Government *spends too little* on pharmaceuticals, it also risks poorer health outcomes from restricting access to the benefits of pharmaceuticals, and also from discouraging the pharmaceutical industry to invest in the research and development of new, more effective medicines.

The aim of a risk management approach to pharmaceutical development, approval and funding is not to eliminate the risks — the risks are an essential element of the research and discovery process, which leads to innovative, more effective pharmaceuticals. From the Government's perspective, the aim is to manage the risk on behalf of the community to attain the benefits, through regulatory processes that encourage pharmaceutical companies to bear the risk of research and development to bring improved products to market, while ensuring the cost to the budget is affordable and sustainable in the long term.

### **Promoting the cost-effective use of pharmaceuticals**

Australia has been at the forefront of implementing measures to promote the cost-effective use of pharmaceuticals. It was the first country to introduce a formal and systematic use of pharmacoeconomic assessment, which is the practice of assessing and comparing both the benefits and costs of a pharmaceutical product. Pharmacoeconomic assessment underpins the listing and pricing of pharmaceuticals on the Pharmaceutical Benefits Scheme (PBS).

The pharmacoeconomic assessment process is reinforced by measures to encourage prescribers and consumers to choose the most cost-effective pharmaceuticals. Particularly important are policies which encourage the use of generic products when the patent for the underlying preparation of a medication has expired.



Suggestions on ways to improve the Government's policies to promote the cost-effective use of pharmaceuticals have tended to focus on extending the current approaches, for example, a system of periodic reviews of the cost effectiveness of listed medications.

### **Risk management strategies**

Although Australia has been at the forefront of innovative policies to promote the cost-effective use of pharmaceuticals, it is important to ensure that its approach can meet the challenges facing the public financing of pharmaceuticals, particularly the changing world of pharmaceutical development and usage.

As noted above, the development of pharmacogenomics is likely to be a very significant influence on the development of new pharmaceuticals, with major implications for the approval, listing and funding of pharmaceuticals. The predicted developments from pharmacogenomics offer both challenges and opportunities for the Australian healthcare system:

- in terms of *challenges*, the PBS is, and will continue to be, under increasing pressure to fund new innovative, targeted and high-cost therapies for the prevention and treatment of previously unmanageable diseases. Developments in pharmacogenomics may represent major advances in prevention and treatment, but they often come at relatively high cost;
- in terms of *opportunities*, these new interventions include 'targeted' therapies that are designed for use in a well-defined targeted group of patients who have specific biological markers. Thus, it should be possible to establish clear criteria for patient eligibility for the new targeted therapies to optimise health outcomes, accurately predict the budgetary implications to government if listed on the PBS, and minimise opportunity for inappropriate prescribing through 'leakage'.

This report puts forward for discussion approaches based on risk sharing among the Government, pharmaceutical companies and healthcare providers, which acknowledge the challenges and opportunities of the changing world of pharmaceuticals. They have the potential to help better manage the risk of funding pharmaceuticals in the context of significant uncertainty about both the benefits and the costs.

For example, a funding model which has emerged in the UK in response to the development of high-cost biotechnology medicines and other targeted therapies is risk sharing based on outcome guarantees. Under an outcomes guarantee, a pharmaceutical company and prescribing stakeholders agree on the outcomes that they would expect from a medication in a given indication. If the medicine fails to fulfil expectations, the pharmaceutical company refunds the health service for the cost of the medicine. This encourages the pharmaceutical company to promote responsible prescribing and ensures that healthcare resources are not wasted on ineffective treatments. Another example of a risk sharing approach to funding is the recent listing of a new treatment for arthritis under the PBS, which was based on an agreement around eligibility criteria, prescribing rights and expected expenditure.

It is too soon to say whether risk-sharing approaches will be successful; furthermore, the UK's scheme for multiple sclerosis drugs has faced many implementation challenges. However, an appropriately framed strategy could help resolve uncertainties about cutting edge treatments where the ultimate impact of costly medications on health outcomes is not known throughout much of the life of their patents, or of the patients taking them. There could be other benefits — there may be faster listing of high-cost medicines with the trade-off being close monitoring of what happens post-listing. This could be a new approach, with the possibility of altering decisions going forward based on what happens to medicines in the field by monitoring real outcomes in real life.

## Chapter 1

# Introduction

The Productivity Commission is undertaking a study detailing the impact of advances in medical technology on healthcare expenditure in Australia, and the associated costs and benefits for the Australian community. The aim of the study is to assist governments and other stakeholders by improving the level of understanding about the relationship between advances in medical technology, health outcomes and health expenditure.<sup>1</sup>

This report responds to the Commission's request for information relevant to the study's terms of reference. It does so from the perspective of one area of medical technology: pharmaceuticals.

The report is structured as follows:

- chapter 2 provides an overview of pharmaceutical use and expenditures in Australia, and looks at the drivers for the future;
- chapter 3 looks at the impact of advances in pharmaceuticals, but from a broader perspective of their costs and benefits than is usual. This includes consideration of the impact on other forms of healthcare expenditure, as well as on the economic and health benefits;
- chapter 4 discusses the role of the Government in promoting the cost-effective use of pharmaceuticals, providing an overview of current approaches and making suggestions for future directions. It highlights the significant level of uncertainty about both the costs and benefits of pharmaceuticals, and the potential of risk-sharing approaches between the Government and pharmaceutical companies that acknowledge the challenges and opportunities of the changing world of pharmaceuticals and seek in that context to maximise the cost effectiveness of pharmaceutical usage and expenditure.

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<sup>1</sup> Productivity Commission 2004, *Impact of Advances in Medical Technology on Healthcare Expenditure in Australia*, Productivity Commission Issues Paper, p. 9.

## Chapter 2

# Pharmaceutical use and expenditures in Australia

### KEY POINTS

There has been a great deal of focus recently on the increase in the use of and expenditure on pharmaceuticals:

- in 2003–04, 181 million subsidised pharmaceuticals were prescribed in Australia — 62 per cent more than the number prescribed in 1992–93;
- real expenditures on pharmaceuticals in Australia grew by an average of 9.5 per cent per year from 1992–93 to 2002–03, reaching \$9.9 billion in 2002–03; and
- expenditures on pharmaceuticals represent an increasing share of total health expenditure, from 9.5 per cent in 1992–93 to 15 per cent in 2002–03.

Rising expenditures on pharmaceuticals are the result of a number of factors, including:

- population growth and ageing;
- increases in the number of pharmaceuticals per person;
- introduction of new therapeutic agents;
- changes in the mix of existing therapies towards more costly and improved agents; and
- increased identification and treatment of medical conditions.

Looking towards the future, these factors are likely to continue to drive increasing expenditure on pharmaceuticals.

Developments in pharmacogenomics — the study of how people's genes affect the way they respond to medicines — also have the potential to lead to innovative therapies for the prevention and treatment of diseases, but at high costs.

It is not simple to assess the appropriateness of the increasing expenditure on pharmaceuticals in Australia. It depends on the nation's preferences, the opportunity costs of the expenditure, and in particular, the cost-effectiveness of the expenditure. This requires a comprehensive understanding of the costs and benefits of pharmaceuticals.

Recent discussions about the impact of advances in medical technology on healthcare expenditure in Australia have had a significant focus on pharmaceuticals. For example, the Intergenerational Report projects that the majority of the increase in Commonwealth healthcare expenditures will be derived from growth in the cost of the Pharmaceutical Benefits Scheme (PBS).<sup>2</sup> More recently, the Productivity Commission's Report on Ageing highlights in the context of the ageing of the population that average PBS costs for a male aged 65–74 years are more than 18 times the average costs for a male aged 15–24 years.<sup>3</sup>

This chapter looks at the information on the use of and expenditure on pharmaceuticals:

- section 2.1 describes trends in the usage of pharmaceuticals, pointing to the increasingly significant role played by pharmaceuticals in the delivery of healthcare in Australia;
- section 2.2 discusses the consequential increase in expenditure on pharmaceuticals, and the factors accounting for the increase; and

<sup>2</sup> Commonwealth of Australia 2002, 'Intergenerational Report 2002-3', Budget Paper No. 5, Canberra.

<sup>3</sup> Productivity Commission 2004, *Economic Implications of an Ageing Australia*, Draft Research Report, Productivity Commission, Canberra, pp. 6.4-6.5.

- section 2.3 discusses the implications of developments in pharmaceuticals for usage and expenditure, particularly the development of pharmacogenomics.

The chapter concludes with the point that it is not simple to assess the appropriateness of the increasing expenditure on pharmaceuticals in Australia, as this requires a comprehensive understanding of both the costs and the benefits of pharmaceuticals.

## **2.1 Australians' use of pharmaceuticals**

### ***Aggregate trends in use***

Australians are using more pharmaceuticals than ever before (figure 2.1). In 2003–04, 181 million 'subsidised' pharmaceutical products were prescribed in Australia under the Pharmaceutical Benefits Scheme (PBS) or the Repatriation Pharmaceutical Benefits Scheme (RPBS). This was 62 per cent (or 69 million) more than the number prescribed eleven years earlier in 1992–93.<sup>4</sup> At the same time, the number of medicines listed on the PBS has risen, climbing by 255 medicines between 1996 and 2004. Today, the PBS subsidises about 650 different medications.<sup>5</sup>

The rate of increase in subsidised pharmaceutical prescriptions has been much higher than the growth in the population, which rose 14 per cent over the same period. In 2003–04, about 9 prescriptions of subsidised pharmaceuticals were being made for every Australian — almost three prescriptions per person more than in 1992–93.

The information available about prescriptions of non-subsidised pharmaceuticals is less extensive than that about subsidised pharmaceuticals. The Department of Health and Ageing has published estimates of prescriptions for non-subsidised pharmaceuticals, based on a survey of the pharmaceutical dispensing records of 150 pharmacies. These data indicate that the number of prescriptions of non-subsidised pharmaceuticals in Australia was reasonably constant at about 45 million prescriptions per year between 1995 and 2000. Over the same period, subsidised prescriptions grew by 21 per cent (figure 2.1). In 2000, almost four of every five pharmaceutical prescriptions in Australia were subsidised by taxpayers.

There are no publicly available data on the pharmaceutical usage rates of different age groups in the population. However the Productivity Commission, using unpublished Health Insurance Commission data, recently reported that the per capita cost of PBS-subsidised pharmaceuticals are strongly age-related, with the average cost for a person aged 65–74 years more than 18 times the average cost for a person aged 15–24 years, and about five times the average cost of a person aged 40–50 years.<sup>6</sup>

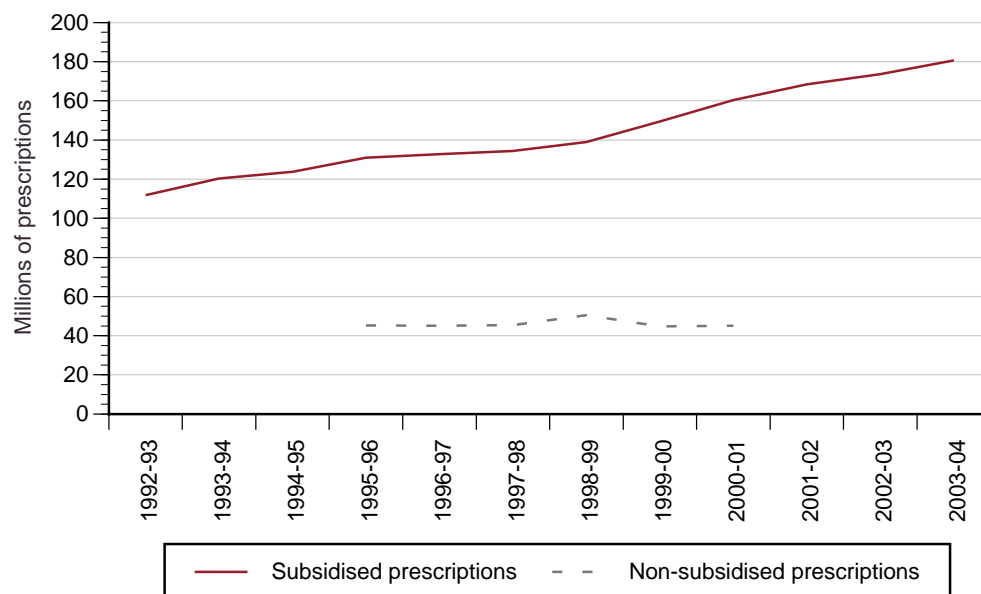
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<sup>4</sup> Health Insurance Commission 2004, *Pharmaceutical Benefits Schedule Group Statistics*, [www.hic.gov.au/statistics/dyn\\_pbs/forms/pbsgtab1.shtml](http://www.hic.gov.au/statistics/dyn_pbs/forms/pbsgtab1.shtml), accessed on 29 November 2004.

<sup>5</sup> Department of Health and Ageing 2004, unpublished data.

<sup>6</sup> Productivity Commission 2004, *Economic Implications of an Ageing Australia*, Draft Research Report, Productivity Commission, Canberra, pp. 6.4–6.5

Figure 2.1

**PRESCRIPTIONS OF PHARMACEUTICALS, AUSTRALIA, 1992–93 TO 2003–04**

Note: 'Subsidised prescriptions' are prescriptions dispensed under the Pharmaceutical Benefits Scheme (PBS) or the Repatriation Pharmaceutical Benefits Scheme (RPBS). 'Non-subsidised prescriptions' are private prescriptions and PBS prescriptions priced under the general patient co-payment. Pharmaceuticals prescribed in public hospitals are excluded from these figures.

Source: Health Insurance Commission 2004, *Pharmaceutical Benefits Schedule Group Statistics*, [www.hic.gov.au/statistics/dyn\\_pbs/forms/pbsgtab1.shtml](http://www.hic.gov.au/statistics/dyn_pbs/forms/pbsgtab1.shtml), accessed on 29 November 2004; and Department of Health and Ageing 2003, *Australian Statistics on Medicines 1999-2000*, AusInfo, Canberra.

**Trends in pharmaceutical categories**

Pharmaceuticals are divided into 14 main anatomical categories under the Anatomical Therapeutic Chemical (ATC) system of classification (figure 2.2). In 2003–04, 63 per cent of subsidised prescriptions in Australia fell into three of these categories:

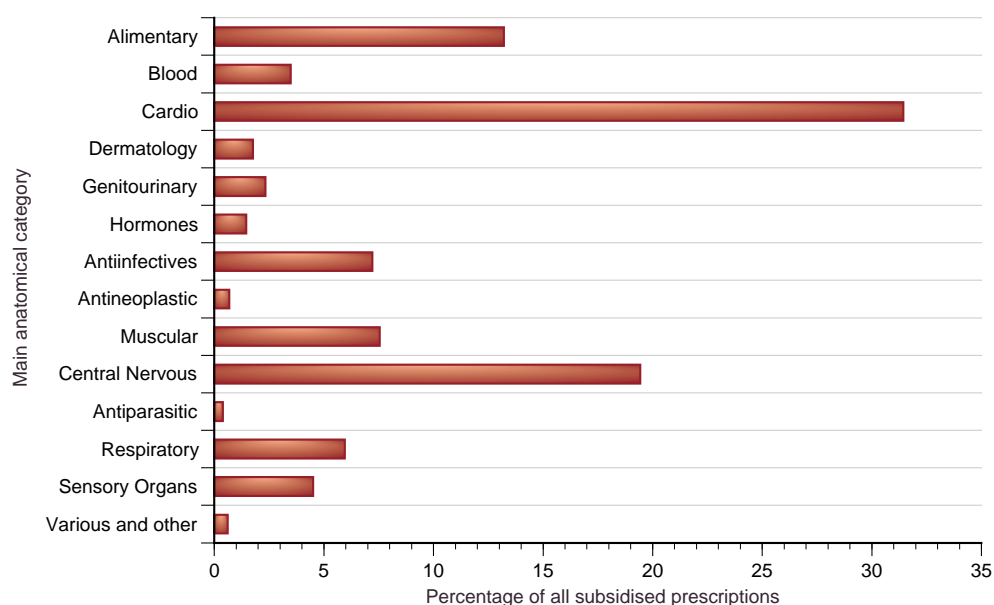
- cardiovascular system (31 per cent);
- central nervous system (19 per cent); and
- alimentary tract and metabolism (13 per cent).

The most commonly prescribed categories of *non-subsidised* pharmaceuticals in 2000 were:

- general anti-infectives for systemic use (24 per cent of non-subsidised prescriptions);
- central nervous system (17 per cent); and
- genitourinary system and sex hormones (16 per cent).

When non-subsidised prescriptions are taken into account, general anti-infectives for systemic use was the third largest category of prescriptions overall in 2000, accounting for 12 per cent of all prescriptions (subsidised and non-subsidised).

Figure 2.2

**PROPORTION OF SUBSIDISED PRESCRIPTIONS IN EACH CATEGORY OF PHARMACEUTICALS, 2003–04**

Source: Department of Health and Ageing 2003, *Australian Statistics on Medicines 1999-2000*, AusInfo, Canberra.

The increase in subsidised prescriptions from 1992–93 to 2003–04 has been heavily concentrated in a small number of categories. Prescriptions of medications for the cardiovascular system rose by 129 per cent, or 32 million — representing 47 per cent of the overall growth in subsidised prescriptions. Growth in medications for the central nervous system (74 per cent), the alimentary tract and metabolism (100 per cent), and the musculo-skeletal system (100 per cent) represented a further 49 per cent of overall growth. These four categories thus contributed 95 per cent of the growth in total prescriptions.

Although cardiovascular medications had the largest growth in absolute terms, the class did not have the largest percentage growth rate. Two other categories had larger proportional increases in subsidised prescriptions between 1992–93 and 2003–04, both rising from a low base. Prescriptions of pharmaceuticals for blood and blood forming organs grew 317 per cent to 6.3 million, and prescriptions of medications for antineoplastic and immunomodulating agents rose 246 per cent to 1.2 million.

There were substantial declines in subsidised prescriptions of three categories of pharmaceuticals:

- genitourinary system decreased by 1.1 million, or 22 per cent, to 4.2 million
- dermatologicals decreased by 840 000, or 21 per cent, to 3.2 million; and
- general anti-infectives for systemic use decreased by 2.5 million, or 16 per cent, to 13 million.

## 2.2 Pharmaceutical expenditure

### *Trends in expenditure*

Together with the rise in the use of pharmaceuticals, there has been an increase in expenditure on pharmaceuticals:

- real expenditure on pharmaceuticals in Australia grew by an average of 9.5 per cent per year from 1992–93 to 2002–03, reaching \$9.9 billion in 2002–03. Growth peaked at 17 per cent in 2000–01;
- annual growth between 1997–98 and 2002–03 was 11.1 per cent, in comparison to annual average growth of 7.1 per cent between 1992–93 and 1996–97;
- pharmaceutical expenditures represented an increasing share of total health expenditures over time, rising from 9.5 per cent in 1992–93 to 15 per cent in 2002–03.<sup>7</sup>

The Australian Government and individuals are the primary sources of funding for pharmaceuticals expenditures (table 3.1). Government outlays arise largely through the Pharmaceutical Benefits Scheme (PBS), a universal scheme that provides subsidised pharmaceuticals. To obtain medicines listed on the PBS, individuals must contribute a co-payment, which varies according to an individual's family structure and concessional status. Non-subsidised pharmaceuticals include medicines on the PBS whose price falls below the statutory patient contribution for the class of patient concerned, medicines not listed on the PBS, and over-the-counter medications, vitamins and medical non-durables which are not subsidised by the Government.

Government outlays are the main source of funding for subsidised pharmaceuticals, accounting for 81 to 85 per cent of total spending in this category. In real terms, expenditures on subsidised pharmaceuticals grew an average of 10.4 per cent per year from 1992–93 to 2002–03, with slightly lower growth experienced in the first five years (9.6 per cent) than in the second six years (10.9 per cent). Growth over the entire period was shared between the Government (10.6 per cent) and individuals (9.7 per cent).

Expenditures on non-subsidised pharmaceuticals grew more slowly than spending on subsidised pharmaceuticals, averaging an annual growth rate of 8.3 per cent between 1992–93 and 2002–03. Average growth was substantially lower between 1992–93 and 1996–97 (3.6 per cent) than it was between 1997–98 and 2002–03 (11.5 per cent). The main sources of funding for non-subsidised pharmaceuticals are individuals' out-of-pocket expenditures. The Government spending from 1997–98 onwards in this category reflects the Government's 30 per cent rebate for private health insurance.

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<sup>7</sup> These figures do not include expenditures for drugs and medical non-durables dispensed in hospitals, which are trivial in comparison to total subsidised and non-subsidised pharmaceutical expenditures. In 2002–03, 2.4 million was spent on hospital pharmaceuticals, up from 1.2 million in 1995–96.



Table 2.1

**PHARMACEUTICAL EXPENDITURES, CONSTANT PRICES, 1992–93 TO 2002–03**

|                                  | Subsidised (\$ millions) |            |             | Non-subsidised (\$ millions) |            |            |
|----------------------------------|--------------------------|------------|-------------|------------------------------|------------|------------|
|                                  | Gov't                    | Indiv      | Total       | Gov't                        | Indiv      | Total      |
| 1992–93                          | 1880                     | 422        | 2302        | 0                            | 1661       | 1730       |
| 1993–94                          | 1929                     | 404        | 2333        | 0                            | 1683       | 1755       |
| 1994–95                          | 2124                     | 469        | 2593        | 0                            | 1821       | 1900       |
| 1995–96                          | 2538                     | 500        | 3038        | 0                            | 1712       | 1812       |
| 1996–97                          | 2749                     | 556        | 3305        | 0                            | 1883       | 1982       |
| 1997–98                          | 2808                     | 599        | 3407        | 3                            | 2208       | 2300       |
| 1998–99                          | 3096                     | 628        | 3724        | 7                            | 2361       | 2451       |
| 1999–00                          | 3528                     | 681        | 4209        | 14                           | 2583       | 2693       |
| 2000–01                          | 4316                     | 775        | 5091        | 81                           | 2805       | 2995       |
| 2001–02                          | 4743                     | 840        | 5583        | 84                           | 3154       | 3365       |
| 2002–03                          | 5063                     | 1045       | 6107        | 58                           | 3693       | 3802       |
| <b>Average annual growth (%)</b> |                          |            |             |                              |            |            |
| 1992–93 to 1996–97               | 10.1                     | 7.4        | 9.6         |                              | 3.4        | 3.6        |
| 1997–98 to 2002–03               | 10.9                     | 11.3       | 10.9        | 136.9                        | 12.0       | 11.5       |
| <b>1992–93 to 2002–03</b>        | <b>10.6</b>              | <b>9.7</b> | <b>10.4</b> |                              | <b>8.5</b> | <b>8.3</b> |

Note: 'Subsidised prescriptions' are prescriptions dispensed under the Pharmaceutical Benefits Scheme (PBS) or the Repatriation Pharmaceutical Benefits Scheme (RPBS). 'Non-subsidised prescriptions' are private prescriptions and PBS prescriptions priced under the general patient co-payment. Total expenditures include outlays by private health insurance funds and state governments not shown. Expenditures on pharmaceuticals prescribed in public hospitals are excluded from these figures.

Source: Australian Institute of Health and Welfare 2004, *Health expenditure Australia 2002–03*, <http://www.aihw.gov.au/dataonline/index.html>, accessed 5 November 2004.

### ***Explaining the increase in pharmaceutical expenditures***

Rising expenditures on pharmaceuticals are the result of a number of factors, including:

- population growth and ageing;
- increases in the number of pharmaceuticals per person;
- introduction of new therapeutic agents;
- changes in the mix of existing therapies towards more costly and often better agents; and
- increased identification and treatment of medical conditions.

The trend in Australia can be better understood by an analysis of these different factors.

Although population growth and ageing contributed to the increasing expenditures, both the number of prescriptions and expenditures per person per year rose over time:

- section 2.1, for example, notes that the number of prescriptions per person per year increased from three to nine between 1992–93 and 2003–04;
- likewise, real per capita expenditures on subsidised pharmaceuticals rose an average of 9.2 per cent per year between 1992–93 and 2002–03 to a high of \$306.77 in 2002–03;
- per person expenditures on non-subsidised pharmaceuticals increased by an annual average of 6.6 per cent during the same time period, reaching \$190.98 in 2002–03;
- per person rates of growth are slightly lower than growth rates for aggregate expenditures on pharmaceuticals (10.4 per cent for subsidised items and 8.3 per cent for non-subsidised items). The difference between the growth rates in each category is the result of growth in the overall size of the Australian population.

Average cost increases in Australia also play an important role in explaining the increase in pharmaceutical expenditures. Between 1992–93 and 2002–03, real total expenditures per subsidised prescription grew by 5.6 per cent per year on average (table 3.2). This growth rate exceeds the 2.9 per cent average annual rate of health inflation during this time.<sup>8</sup> In 2002–03, the average cost of a subsidised pharmaceutical was \$35.18 (in 2000–01 dollars), up from \$25.35 in 1997–98 and \$20.59 in 1992–93.

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<sup>8</sup> Australian Institute of Health and Welfare (AIHW) 2004, *Health expenditure Australia, 2002-03*, AIHW Cat. No. HWE 27, Canberra.

Table 2.2

**AVERAGE EXPENDITURE PER SUBSIDISED PHARMACEUTICAL, CONSTANT PRICES, 1992–92 TO 2002–03**

|                                  | Amount (\$2000–01) | Growth rate (%) |
|----------------------------------|--------------------|-----------------|
| 1992–93                          | 20.59              |                 |
| 1993–94                          | 19.38              | –5.9            |
| 1994–95                          | 20.95              | 8.1             |
| 1995–96                          | 23.21              | 10.8            |
| 1996–97                          | 24.90              | 7.3             |
| 1997–98                          | 25.35              | 1.8             |
| 1998–99                          | 26.79              | 5.7             |
| 1999–00                          | 28.17              | 5.1             |
| 2000–01                          | 31.74              | 12.7            |
| 2001–02                          | 33.14              | 4.4             |
| 2002–03                          | 35.18              | 6.2             |
| <b>Average annual growth (%)</b> |                    |                 |
| 1992–93 to 1996–97               |                    | 5.1             |
| 1997–98 to 2002–03               |                    | 5.9             |
| <b>1992–93 to 2002–03</b>        |                    | <b>5.6</b>      |

Note: 'Subsidised prescriptions' are prescriptions dispensed under the PBS or the RPBS.

Source: HIC 2004, *Pharmaceutical Benefits Schedule Group Statistics*,

[www.hic.gov.au/statistics/dyn\\_pbs/forms/pbsgtab1.shtml](http://www.hic.gov.au/statistics/dyn_pbs/forms/pbsgtab1.shtml), accessed on 29 November 2004; and AIHW 2004, *Health expenditure Australia 2002–03*, <http://www.aihw.gov.au/dataonline/index.html>, accessed 5 November 2004; and ABS 2003, *Population Statistics*, Ausinfo, Canberra.

### 2.3 Future role of pharmaceuticals in delivery of healthcare

Technological advances are largely responsible for the increasing use of pharmaceuticals in the last two decades, a trend that is likely to continue. The future role of pharmaceuticals in the delivery of healthcare will also depend on changes in the demographic composition of Australia and interactions between the factors underlying the use of medicines.

#### *Future pharmaceutical discoveries*

In the medium term, the application of *pharmacogenomics* — the study of how people's genes affect the way they respond to medicines — is likely to create the greatest range of new possibilities for pharmaceuticals. By identifying genes that determine whether certain individuals are likely to have positive (or negative) reactions to particular medicines, scientists will be able to design simple genetic tests that determine in advance which medications will work for a given individual and which will not — both in terms of the avoidance of adverse events and increased efficacy.

The refined understanding of how medicines interact with individuals' genetic compositions learned through pharmacogenomics can also be used to identify which medications are the best candidates for clinical trials. Applications could also identify and exclude from research trials those individuals who are less likely to respond or are at risk of adverse reactions at later stages of clinical trials. Better targeting in this field could increase the reliability of scientific findings by using smaller groups of genetically similar participants in clinical trials, as well as shorten the average time to develop a medication, potentially reducing research and development costs.

In the longer term, pharmacogenomics may allow researchers to tailor medicines to different segments of the population with a particular type of genetic composition. The incorporation of this type of development into clinical practice has the potential to improve efficacy and reduce toxicity by allowing the choice of the 'right medication for the right patient in the right disease at the right dose'.<sup>9</sup> Some predicted developments from pharmacogenetic research are set out in box 2.1.

Box 2.1

**EXAMPLES OF THE PREDICTED USE OF PHARMACOGENOMICS**

- Establishment of prescribing guidelines, based on clinical studies, for medications that are subject to substantial polymorphic metabolism.
- Prescribing advice will relate dose to genotype and will highlight the possibility of medication interactions when multiple medications are prescribed concomitantly.
- Establishment and recording of individual patient genotypes — that is, 'personal pharmacogenetic profiles'.
- Pharmacogenetic testing will reduce the need for hospitalisation, and its associated costs, because of adverse medication reactions.
- Development of new medications for patients with specific genotypes — that is, 'medication stratification'.

Source: C. Wolf, G. Smith and R. Smith 2000, 'Science, medicine, and the future: Pharmacogenomics', *British Medical Journal*, vol. 320, 8 April, p. 987.

In addition to pharmacogenomics, there are many other research fields being explored by scientists to discover new and innovative medicines. Predicted pharmaceutical discoveries include:

- medicines that prevent the HIV virus from entering a cell at the outset;<sup>10</sup>
- medications that slow the progression of Alzheimer's disease;<sup>11</sup>
- combinations of treatments that cut off the blood supply to cancerous tumours;<sup>12</sup>
- medicines that prompt the heart to grow new blood vessels, providing the same benefits currently delivered by bypass surgery;<sup>13</sup>

<sup>9</sup> M. Pirmohamed and G. Lewis 2004, 'The implications of pharmacogenetics and pharmacogenomics for drug development and healthcare', in E. Mossialos, M. Mrazek and T. Walley (eds), *Regulating Pharmaceuticals in Europe: Striving for Efficiency, Equity and Quality*, Open University Press, Berkshire, p. 281.

<sup>10</sup> *Time* 2001, 'The Hunt for Cures', 15 January.

<sup>11</sup> *Ibid.*

<sup>12</sup> *Ibid.*

<sup>13</sup> *The Wall Street Journal* 2000, 'Next Milestones in Human Genetics', 26 May.

- treatments that regenerate nerves damaged by brain disease or spinal cord injury;<sup>14</sup> and
- multi-component fixed dose combination medicines that prevent cardiovascular disease.<sup>15</sup>

### **Demographic changes**

Australia's ageing population is likely to play a large role in the use of pharmaceuticals in the next two decades. The increase in the absolute size of the elderly population is likely to impact the demand for pharmaceuticals.

### **Factors driving future use of pharmaceuticals**

Current and future demand for pharmaceuticals differs from the demand for standard consumption goods. In particular, the consumer of medicines typically does not decide how much and which product to use. At the same time, for many pharmaceutical products — particularly prescribed medications — the consumer does not pay the full price of the good. These features mean that pharmaceutical use is determined by a number of interacting factors.

*Consumers' expectations* and preferences for pharmaceutical intervention impact current and future use. These opinions are based on the degree and quality of available information about the efficacy and accessibility of treatments. The breadth of information about health conditions and treatments published through the Internet has provided consumers with an increasing amount of easily accessible information. Preferences for pharmaceuticals also depend on their associated side effects and perceived ease of use. Medicines for acquired immunodeficiency syndrome (AIDS) provide an example of improvements in ease of pharmaceutical use. In contrast to the first treatments that had to be taken multiple times a day, current medications are available in once or twice daily forms.<sup>16</sup>

The *discovery of new therapies* and the continued *commitment of pharmaceutical manufacturers* to innovate also play a role in the patterns of use. In 2002, for example, researchers investigated 28 per cent more medicines for approval than a decade earlier. However, innovation is complex and comes at a high cost to developers. Developing, testing, and gaining approval for a new medication is costly, time consuming and risky. On average, it takes 12 to 15 years to develop and test a new medicine. Further, only five of every five thousand pharmaceuticals developed are tested in clinical trials.<sup>17</sup>

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<sup>14</sup> *Business Week* 1998, 'Biotech Bodies', 27 July.

<sup>15</sup> World Health Organization (WHO) 2004, *Priority Medicines for Europe and the World*.

<sup>16</sup> Pharmaceutical Research and Manufacturers of America (PhRMA) 2003, *A Decade of Innovation*. Available at [www.phrma.org/publications/publications/2003-10-16.855.pdf](http://www.phrma.org/publications/publications/2003-10-16.855.pdf), Accessed 5 November 2004.

<sup>17</sup> Pharmaceutical Research and Manufacturers of America (PhRMA) 2000, *Why do Prescription Drugs Cost so Much... and Other Questions about Your Medicines*, <http://www.phrma.org/publications/publications/brochure/questions/questions.pdf>, Access 5 November 2004.

*Standards of medical care and diagnostic abilities* have a substantial influence on the use of pharmaceuticals and developments in these areas are likely to encourage increased future use. New technologies in diagnostic techniques have enabled providers to identify easily chronic diseases that can be managed with pharmaceuticals.<sup>18</sup> In addition, many guidelines are being revised to favour pharmacological intervention. For example, in 2003, an expert panel updated the guidelines for treating patients with high blood pressure. The new guidelines expand the eligibility criteria for medicinal therapy and increase the number of individuals who need to control their blood pressure. According to the chairman of the committee that produced the new guidelines:

Though improved, the treatment and control rates are still too low. The new guidelines zero in on this problem, recommending factors that often lead to inadequate control such as not prescribing sufficient medication.

## 2.4 Conclusions

This chapter began by noting that recent discussions about the impact of advances in medical technology on healthcare expenditure in Australia have had a significant focus on pharmaceuticals. This is understandable as the data show increasing usage of pharmaceuticals with an associated significant increase in expenditure. The factors underpinning the increase in usage and expenditure — including developments in technology, the ageing of the population and consumer preferences — are likely to continue and even strengthen with the development of pharmacogenomics.

It is not simple to assess the appropriateness of the increasing expenditure on pharmaceuticals in Australia. It is not simple to judge whether the expenditure is too little, too much or just right. It depends on the nation's preferences, the opportunity costs of the expenditure, and in particular, the cost effectiveness of the expenditure — taking into account the total (net) effects on costs across the health system as a whole, not simply the cost of pharmaceuticals in isolation. This requires a comprehensive understanding of both the costs and the benefits of pharmaceuticals. This issue is discussed in chapter 3.

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<sup>18</sup> K. Harvey 2002, *Securing the Future of the Pharmaceutical Benefits Scheme?*, Australian Review of Public Affairs (ARPA) Symposium: The 2002-03 Federal Budget.

<sup>19</sup> National Heart, Lung and Blood Institute 2003, 'NHLBI Issues New High Blood Pressure Clinical Practice Guidelines', press release, available at [www.nhlbi.nih.gov/new/press/03-05014.htm](http://www.nhlbi.nih.gov/new/press/03-05014.htm).

## Chapter 3

# Impact of advances in pharmaceuticals: a broader assessment

### KEY POINTS

Predominantly, there has been a fairly narrow approach to the assessment of the costs and benefits of pharmaceuticals.

On the cost side, expenditure on pharmaceuticals should be considered not in isolation but as part of overall resource use in healthcare. From this perspective, expenditure on pharmaceuticals may often be a substitute for, and hence reduce, other healthcare expenditure. It is the effect of pharmaceutical use on net cost of healthcare in total that needs to be weighed up against the benefits.

On the benefit side, in addition to positive impacts on health outcomes, pharmaceuticals can bring about economic benefits by reducing productivity losses due to poor health.

A case study modelling the improved take-up of pharmaceuticals for the treatment and prevention of heart disease shows a significant reduction in expenditure on hospital care, pointing as well to a likely increase in workforce participation for older people at risk of heart disease. Studies of this kind shed a rather different light on the issue than those that focus narrowly on the cost of pharmaceutical (or other medical technology) use.

Recent discussions about the impact of advances in pharmaceuticals have tended to take a fairly narrow approach to the assessment of their costs and benefits. Considering the expenditure on pharmaceuticals separately instead of as part of overall resource use in the healthcare system has been criticised as reflecting a ‘silo mentality’.<sup>20</sup> One disadvantage of such a narrow approach to the assessment of the costs of pharmaceuticals is that it could result in inefficiency because pressure to reduce the consumption of pharmaceuticals could lead to increased consumption of other healthcare resources.

Similarly, the benefits of pharmaceuticals are often described in terms of direct health outcomes, whereas a broader appreciation of the benefits, including, for example, the impact on workforce participation due to healthier employees, allows a more comprehensive view.

This chapter takes a more comprehensive approach to the assessment of the costs and benefits of pharmaceuticals:

- section 3.1 looks at how the cost of pharmaceuticals must be understood in light of the impact on — or substitution for — other health expenditure;
- section 3.2 discusses the benefits of pharmaceuticals from the perspectives of both health outcomes and broader economic benefits; and
- section 3.3 presents the results of a modelling exercise of the impact of healthcare expenditure of improved take-up of pharmaceuticals for the treatment and prevention of heart disease.

<sup>20</sup> M. Drummond 2003, ‘Moving beyond the drug budget silo mentality in Europe’, *Value in Health*, volume 6, supplement 1, S74-S77, p. S74.

### 3.1 Pharmaceuticals as a substitute for other healthcare services

Advances in pharmaceuticals are associated with a change in the way healthcare is delivered, with pharmaceuticals often substituting for other medical interventions. One medical expert describes this phenomenon as part of a permanent change in the way health is maintained:

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 labour to capital.<sup>21</sup>

Several researchers have evaluated the cost effectiveness of pharmaceuticals in light of their substitution for other medical services, as well as their impact on health outcomes. In an examination of five disease areas, Mark McClellan and David Cutler found that the benefits of medical technology, particularly pharmaceuticals and diagnostic techniques, outweighed the costs in four areas and equalled the costs in the fifth area.<sup>22</sup> Similarly, a review of 85 economic evaluations published between 1986 and 1991 found that a medicinal intervention was almost always more cost effective than no intervention in the treatment or prevention of a disease, and in a number of the studies examined, pharmaceutical interventions were at least as cost effective as other forms of medical intervention.<sup>23</sup>

Box 3.1 summarises the results of a sample of studies that seek to quantify the relationship between pharmaceuticals and other types of medical care.

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<sup>21</sup> J.D. Kleinke 2001, 'The Price of Progress: Prescription Drugs in the Healthcare Market', *Health Affairs*, vol. 20, no. 5, pp. 43-60.

<sup>22</sup> M. McClellan and D. Cutler 2001, 'Is technological change in medicine worth it?', *Health Affairs*, vol. 20, no. 5, pp. 11-29.

<sup>23</sup> D. Coyle and M. Drummond 1993, 'Does expenditure on pharmaceuticals give good value for money?: current evidence and policy implications', *Health Policy*, vol. 26, no. 1, pp.55-75.



Box 3.1

**RELATIONSHIP BETWEEN PHARMACEUTICAL AND OTHER MEDICAL EXPENDITURE****Newer medicines versus older medicines**

An increase of US\$18 in spending on new prescription medicines reduces other medical spending by about US\$71, the majority of which is accounted for by reduced hospital expenses. Overall, net savings are US\$53.<sup>24</sup>

**Prescriptions versus hospital days**

An increase of 100 prescriptions is associated with 16.3 fewer hospital days. Translated into dollars, an increase of US\$1 in pharmaceutical expenditures is associated with a US\$3.65 reduction in hospital expenditures.<sup>25</sup>

**Pharmaceuticals versus hospital beds and length of stays**

In the United Kingdom, pharmaceutical use since the 1950s has helped to halve the number of hospital beds used in 12 disease areas and reduce the average length of hospital stays from 45 to eight days. Related savings were estimated at £10 billion per year, or twice the amount spent by the National Health System (NHS) on all medicines.<sup>26</sup>

**3.2 Impact of pharmaceuticals on economic and health outcomes**

Advances in the pharmaceutical industry have played a major role in the prevention, management and treatment of disease. In addition to beneficial impacts on health outcomes, such advances can bring about economic benefits by reducing productivity losses due to poor health.

**Health outcomes**

Pharmaceutical innovation has led to expanded life expectancies, improved health status and a higher quality of life for people. Innovations in medications for asthma provide one of the many examples of this in Australia, where the disease affects almost 15 per cent of the population. Sufferers of this condition experience limited social functioning, poor general health, hospitalisations, lost time from work and premature death.<sup>27</sup> However, new medications for asthma have resulted in a 28 per cent decline in mortality for this group in the last decade.<sup>28</sup>

<sup>24</sup> F. Lichtenberg 2001, 'Are the Benefits of Newer Drugs Worth their Cost?', *Health Affairs*, vol 20, no. 5, p.241-251.

<sup>25</sup> F. Lichtenberg 1996, 'Do (more and better) drugs keep people out of hospitals', *American Economic Review*, vol 86, pp. 384-388.

<sup>26</sup> Association of the British Pharmaceutical Industry (ABPI) 1997, White Paper: ABPI Statement, Available at [http://www.abpi.org.uk/press/press\\_releases\\_97/971210.asp](http://www.abpi.org.uk/press/press_releases_97/971210.asp), Accessed on 4 November 2004.

<sup>27</sup> M. Masoli et al 2003, *Global Burden of Asthma Report*, Medical Institute of new Zealand and University of Southampton (UK), developed for the Global Initiative for Asthma.

<sup>28</sup> A.J. Woolcock et al 2001, 'The Burden of Asthma in Australia', *The Medical Journal of Australia*, vol 175, pp. 141-45.

Several recent studies have compared the impact on health outcomes of existing medicines to new medicines. One such study highlights the association between advances in medicines and morbidity reductions, finding that people who used newer medications had better post-treatment health than people who used older medications for the same condition, controlling for pre-treatment health, age, gender, race, marital status, education, income, and insurance coverage. In particular, individuals taking newer medications reported higher perceived health status and quality of life and they experienced fewer activity and social limitations and more substantial improvements in physical ability.<sup>29</sup>

Pharmaceutical advances have also led to reductions in mortality. For example, pharmaceutical discoveries have eliminated or controlled many diseases and conditions that were once associated with high mortality rates, such as influenza, polio, measles and diphtheria. Results from a study of 52 countries suggest that the launches of new medications account for 40 per cent (or 0.8 years) of the average increase in longevity experienced between 1986 and 2000.<sup>30</sup> Moreover, a recent investigation suggests that pharmaceutical advances will continue to reduce disease-related mortality for years to come. By 2015, pharmaceutical advances are predicted to account for:

- 19 to 40 per cent of the projected reduction in coronary heart disease;
- 28 to 65 per cent of the projected reduction in breast cancer; and
- 15 to 40 per cent of the projected reduction in cerebrovascular disease.<sup>31</sup>

In addition to evidence on the positive health outcomes related to advances in pharmaceuticals in general, there are case studies illustrating the effect of particular medications on certain populations. A sample of these studies is highlighted in box 3.2.

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<sup>29</sup> F. Lichtenberg and S. Virabhak 2002, 'Pharmaceutical Embodied Technical Progress, Longevity, and Quality of Life: Drugs as "Equipment for your Health"' *NBER Working Paper* 9351.

<sup>30</sup> F. Lichtenberg 2003, 'The Impact of New Drug Launches on Longevity: Evidence from Longitudinal, Disease-level Data from 52 Countries, 1982-2001', *NBER Working Paper* 9754.

<sup>31</sup> M. Hall 1994, 'The Impact of Behavioural Advance on Health Trends Over the Next 25 years', *Office of Health Economics Briefing*, no 31, London, UK: Office of Health Economics.

## Box 3.2

**IMPACT OF PHARMACEUTICALS ON DISEASE-SPECIFIC HEALTH OUTCOMES****HIV/AIDS**

Although a cure for HIV/AIDS has yet to be discovered, new medicines have lengthened life expectancy for individuals with the virus and dramatically improved the quality of their lives by enabling them to remain in good health. A recently developed class of medications in this area known as 'fusion inhibitors' effectively prevents the virus from infecting certain components of the immune system. As a result, the amounts of the virus in the bloodstreams of individuals taking these medicines have been reduced to undetectable levels.<sup>32</sup>

**Cancer**

Advances in pharmaceuticals have improved the quality of lives for individuals with cancer by ameliorating some of the adverse effects of chemotherapy. There are now medicines to prevent the associated nausea, restore the energy that is frequently lost with the therapy, and stimulate the often-weakened immune systems of individuals undergoing treatment.<sup>33</sup> At the same time, researchers are discovering medicines that can target cancer cells without damaging healthy cells. An individual involved in a clinical trial of one of these medicines noted

From crawling across the floor on my knees to go to the bathroom, I'm now back at work ... This drug is the magic pill people have dreamed of. It's given me the ability not just to survive, but to have my life back.<sup>34</sup>

**Mental illness**

Pharmaceutical advances have helped transform mental illness from a misunderstood cause of shame into an easily treatable condition. Medicines for depression help individuals with this disease be productive at work, care for their families, and engage in life. Breakthrough advances in pharmaceuticals for schizophrenia have enabled most patients to be treated outside the hospital, not only reducing medical costs, but also lessening the burden of treatment on the patient and family members.<sup>35</sup>

In short, the impact of pharmaceuticals on better health outcomes translates directly into an improved quality of life for countless individuals — not only those suffering from illness but also their carers. This underscores the advantages to individuals of pharmaceutical innovation that accompany the societal benefits. Although it is difficult to put a value on the quality of life for a given person or group of people, such wellbeing is partially reflected also in positive effects on the economy, which are discussed below.

**Economic benefits**

Advances in pharmaceuticals bring about economic improvements by reducing productivity losses due to disease and allowing individuals to retire at older ages. Employers benefit from reduced absenteeism and overall improvements in employees' health; similarly, the Government benefits through the reduced outlays on welfare and the higher tax base associated with a more productive workforce.

<sup>32</sup> Pharmaceutical Research and Manufacturers of America (PhRMA) 2003, *A Decade of Innovation*. Available at [www.phrma.org/publications/publications/2003-10-16.855.pdf](http://www.phrma.org/publications/publications/2003-10-16.855.pdf), Accessed 5 November 2004.

<sup>33</sup> Pharmaceutical Research and Manufacturers of America (PhRMA) 2001, *The Value of Medicines*. Available at [www.phrma.org/publications/publications/value2001/value2001.pdf](http://www.phrma.org/publications/publications/value2001/value2001.pdf), Accessed 5 November 2004.

<sup>34</sup> M. LEMONICK and A. PARK 2001, 'New Hope for Cancer', *Time*, 28 May.

<sup>35</sup> Pharmaceutical Research and Manufacturers of America (PhRMA) 2001, *op. cit.*

A recent study examined the likelihood and impact of a greater number of older Australians staying in the labour force due to improved health. Using a dynamic microsimulation model, it found that in a scenario of improved health in 1998, an additional 500 000 persons aged 65 to 70 years would have remained in the workforce and their earnings would have totalled \$21 billion. The related savings on the age pension would have been almost \$3 billion.<sup>36</sup>

Several studies provide disease-specific evidence of the positive impact of pharmaceutical intervention on productivity:

- Individuals who received an influenza vaccine had 18 to 43 per cent fewer lost workdays and 18 per cent fewer days of reduced effectiveness than individuals receiving a placebo.<sup>37 38</sup>
- Migraine medications were shown to reduce productivity loss by 49 percent per headache during the workday.<sup>39</sup>
- For individuals with diabetes, individuals taking the medicine glipazide lost 5 days per 500 workdays in comparison to their counterparts taking a placebo who lost 24 day during the same length of time.<sup>40</sup>
- The employment rates of persons with schizophrenia doubled with the use of a new atypical antipsychotic medication.<sup>41</sup>
- In a study of employees with depression, anxiety, migraines, or hypertension, researchers found that employees who received pharmaceutical interventions for their condition were able to significantly increase the number of hours worked after treatment from their pre-treatment level.<sup>42</sup>

### 3.3 Case study: advances in pharmaceuticals for cardiovascular disease

An examination of pharmaceuticals developed for cardiovascular diseases provides a practical example of how pharmaceuticals support good health outcomes, prevent adverse health events, and consequently reduce other healthcare expenditures. This example highlights that the net impact on all health expenditures arising from well-targeted increases in the use of pharmaceuticals is substantially less than the gross impact on pharmaceutical expenditures alone.

<sup>36</sup> A. Walker 2004, 'Impact of Health on the Ability of Older Australians to Stay in the Workforce – with Possible Contributions to Economic Sustainability', paper presented at the 'A Future that Works: Economics, Employment and the Environment' Conference, University of Newcastle, Australia December 2004.

<sup>37</sup> H. Rubin 2003, 'Influenza vaccination: not just for those at high risk', *Healthcare Productivity*, National Pharmacy Council, pp5-6

<sup>38</sup> K. Nichol, K. Mallon, and P. Mendelman 2003, 'Cost benefit of influenza vaccination in healthy, working adults: an economic analysis based on the results of a clinical trial of trivalent live attenuated influenza virus vaccine', *Vaccine*, vol. 21, no. 17-18, pp. 2216-2226.

<sup>39</sup> R. Cady 1999, 'Reduction of labor costs associated with treating migraines in the workplace' [editors correspondence], *Archives of Internal Medicine*, vol 159, no. 2, p. 197.

<sup>40</sup> M.A. Testa and D.C. Simonson 1998, 'Health economic benefits and quality of life during improved glycemic control in patients with type 2 diabetes mellitus: a randomised, controlled, double-blind trial', *JAMA*, vol. 280, no. 17, pp. 1490-1496.

<sup>41</sup> W. M. Glazer 1998, 'Formulary decisions and health economics', *Journal of Clinical Psychiatry*, vol. 59, suppl. 19, pp. 23-29.

<sup>42</sup> E. Berndt et al 1997, 'Illness and Productivity: Objective Workplace Evidence', Massachusetts Institute of Technology (MIT), Working Paper #42-97.

### **Cardiovascular disease in Australia**

Cardiovascular diseases affected 3.67 million Australians in 2002, resulting in 50 294 deaths (37.6 per cent of all deaths) in the same year. Further, they were involved in 9.8 per cent of all hospitalisations in 2001–02, the majority of which were experienced by middle-aged and older persons.<sup>43</sup>

Cardiovascular diseases were also the most expensive diseases in terms of health expenditures, accounting for 11 per cent of total health system expenditures — \$5.48 billion in 2000–01. Of this, 46 per cent was spent on hospital care, 26 per cent funded pharmaceuticals, and 14 per cent went towards outpatient services.<sup>44</sup>

### **Impact of cardiovascular medicines on reduced healthcare expenditures**

There is a lot of information supporting the cost effectiveness of cardiovascular pharmaceuticals for patients with heart disease. The following list summarises several studies showing reductions in healthcare expenditures associated with the use of pharmaceuticals to treat cardiovascular disease.

- An Australian study found that the use of pravastatin to lower cholesterol in patients with established heart disease is associated with a 20 per cent reduction in hospital admissions.<sup>45</sup>
- Early initiation of treatment with statins following an acute heart attack reduces the risk of fatal heart disease or a recurrent heart attack by 24 per cent.<sup>46</sup>
- Use of glycoprotein inhibitors reduces the risk of death, a second heart attack, or revascularization by about 50 per cent in patients who had suffered a heart attack previously.<sup>47</sup>
- Adding spironolactone to standard treatments for congestive heart failure reduced deaths by 30 per cent and cut hospitalisations by 35 per cent.<sup>48</sup>
- Use of a cholesterol-lowering agent cut hospitalisations by one-third over five years, reduced the number of days that patients had to spend in hospital when they were admitted, and reduced the need for bypass surgery and angioplasty.<sup>49</sup>

<sup>43</sup> Australian Institute of Health and Welfare (AIHW) and National Heart Foundation of Australia 2004, *Heart, stroke and vascular diseases – Australian Facts 2004*, AIHW Cat. No. CVD 27, Canberra.

<sup>44</sup> Australian Institute of Health and Welfare (AIHW) 2004, *Health system expenditure on disease and injury in Australia, 2000-01*, AIHW Cat. No. HWE 26, Canberra.

<sup>45</sup> P. P. Glasziou et al 2002, 'Cholesterol-lowering therapy with pravastatin in patients with average cholesterol levels and established ischaemic heart disease: is it cost-effective?' *Medical Journal of Australia*, vol. 177: pp. 420-426.

<sup>46</sup> F. M. Sacks et al 1996, 'The effect of pravastatin on coronary events after myocardial infarction in patients with average cholesterol levels', *New England Journal of Medicine*, vol. 335, pp. 1001-09.

<sup>47</sup> M. S. Sabatine and I. K. Jang 2000, 'The use of glycoprotein IIb/IIIa inhibitors in patients with coronary artery disease', *American Journal of Medicine*, vol. 109, pp. 224-37.

<sup>48</sup> B. Pitt et al 1999, 'The Effect of Spironolactone on Morbidity and Mortality in Patients with Severe Heart Failure', *New England Journal of Medicine*, vol. 341, no. 10, pp. 709-17.

<sup>49</sup> *New York Times* 1995, 'Cholesterol Pill Linked to Lower Hospital Costs', 27 March.

### **Future use of pharmaceuticals for cardiovascular disease**

Despite the evidence of the positive impacts of pharmaceuticals on the health outcomes of individuals with cardiovascular diseases, many members of the medical community claim this treatment strategy is under-used. A recent report by the World Health Organization on the use of medicines in Europe notes that the use of proven secondary prevention strategies for cardiovascular disease is substantially incomplete, with a large number of patients going without treatment that would significantly reduce their risks of future heart attack, stroke or death.<sup>50</sup>

Supporting this view, a 1999–2000 study of more than three thousand patients who had been admitted with ischaemic heart disease to about 50 hospitals in Europe found that only 66 per cent of the patients received beta-blockers, 43 per cent received ACE-inhibitors, and 63 per cent were administered cholesterol lowering therapy. Ideally, all patients should have received all treatments.<sup>51</sup> Likewise, evidence from the United States suggests that only one-third of the individuals who should take statins according to current guidelines take them.<sup>52</sup>

This perceived inadequacy in the use of medicines for cardiovascular disease is supported by a belief amongst many medical experts that cholesterol-lowering medicines, particularly statins, should be used as primary prevention for everyone, or at least for a considerably wider group than those with already evident symptoms. This view is supported by a breakthrough study in the United States showing that an intensive statin regimen provides greater protection against death or major cardiovascular events than a standard regimen. In other words, it shows that lowering cholesterol to very low levels is better than previously thought.<sup>53</sup>

Researchers who designed a single daily pill to prevent cardiovascular disease with minimal adverse effects drew similar conclusions. Upon formulating and testing this pill, the study claims:

The Polypill strategy could largely prevent heart attacks and stroke if taken by everyone aged 55 and older and everyone with existing cardiovascular disease. It would be acceptably safe and with widespread use would have a greater impact on the prevention of disease in the Western world than any other single intervention.<sup>54</sup>

### **Estimating the impact of pharmaceutical advances on future health expenditures**

It is difficult to make accurate and meaningful predictions of the impact of pharmaceutical advances on future healthcare expenditures. However, it is possible to employ a basic modelling exercise to understand how an isolated change in pharmaceutical technology could impact future spending. The idea behind this exercise is to compare the projected future outcomes on healthcare expenditures under a base case that reflects the current situation to a hypothetical case that involves technological change.

<sup>50</sup> B. Neal 2004, *Secondary Prevention of Cardiovascular Disease: Fixed Dose Combinations, A Research Agenda for the European Union*, prepared for World Health Organization 'Priority of Medicines for Europe and the World' project.

<sup>51</sup> EUROASPIRE I and II Group 2000, 'Clinical reality of coronary prevents guidelines: a comparison of EUROASPIRE I and II nine countries', *Lancet*, vol 357, pp. 996-1001.

<sup>52</sup> R. Stein 2004, 'Cholesterol drug study finds higher doses help: Aggressive statin use can cut strokes, death', *Washington Post*, 9 March.

<sup>53</sup> C. P. Cannon et al 2004, 'Intensive versus Moderate Lipid Lowering with Statins after Acute Coronary Syndromes', *New England Journal of Medicine*, vol. 350, no. 15, pp. 1495-1504.

<sup>54</sup> N.J. Walk and M.R. Law 2003, 'A strategy to reduce cardiovascular disease by more than 80%', *British Medical Journal*, vol 326, pp 1419-25.

Considering the current debate on medicinal interventions for cardiovascular disease, one potential hypothetical scenario involves an expanded use of medications for treatment and prevention of this disease. More specifically, the hypothetical case is defined by an increase in the use of cardiovascular pharmaceuticals among individuals 55 years and older. The findings in the literature suggest that an increase in the use of pharmaceuticals for cardiovascular disease of this magnitude would be associated with a reduction in hospital episodes, among other benefits. Box 3.3 defines the base and hypothetical case in more detail.

Box 3.3

#### BASE AND HYPOTHETICAL CASES FOR FUTURE EXPENDITURES PROJECTIONS

##### Base case

- Cardiovascular pharmaceutical use as observed in 2002; assume this rate of use is half of what it should be for individuals 55 years and older.
- Hospital episodes and expenditures are as observed in 2002.

##### Hypothetical case: expanded use

- Assume use of cardiovascular pharmaceuticals by individuals 55 years and older is twice the rate observed in base case.
- Hospital episodes for individuals 55 or older are consequently reduced by 20 per cent; hospital expenditures based on lower levels of episodes.

Future healthcare expenditures arising in the base and hypothetical cases are projected by the *Model of Australian Healthcare Episodes, Costs and Funding*.<sup>55</sup> This model projects the future of the present Australian health system, incorporating key sources of pressure on the health system, including:

- the future evolution of the demand for/provision of episodes of care, healthcare costs and sources of funding; and
- an attribution of the projected changes to their main drivers (ageing, usage per capita, input cost movements compared to wider economy cost movements, scenarios for changed technology, treatment or funding structures, etc).

Table 3.1 shows the results of this modelling exercise, listing the difference in expenditures on hospital episodes and subsidised and non-subsidised pharmaceuticals for cardiovascular disease between the hypothetical and base cases. It shows that the increase in PBS and non-PBS expenditures on medications for cardiovascular disease is substantially offset by a decrease in expenditures at public and private hospitals. For example, it is projected that under the hypothetical case, there would be an additional \$1.7 billion in pharmaceutical expenditures in 2006, but when the reduction in hospital expenditures is taken into account, the net impact on health expenditures is only \$224 million — only 13 per cent of the gross increase in pharmaceutical expenditures. By 2015, the net cost grows to \$737 million, or 23 per cent of the gross increase in pharmaceutical expenditures.

<sup>55</sup> The data used in the model were last comprehensively updated in 2002. See [http://www.allenconsult.com.au/publications\\_healthforum.php](http://www.allenconsult.com.au/publications_healthforum.php) for more information on the model.



Table 3.1

**DIFFERENCE IN EXPENDITURES BETWEEN HYPOTHETICAL AND BASE CASE, 1999 PRICES (\$MILLIONS)**

|      | <b>PBS<br/>Cardio</b> | <b>Non PBS<br/>Cardio</b> | <b>Public &amp;<br/>Private<br/>Hospitals</b> | <b>Net Change</b> |
|------|-----------------------|---------------------------|---|-------------------|
| 2006 | 1475                  | 238                       | -1488   | 224               |
| 2007 | 1579                  | 255                       | -1571   | 263               |
| 2008 | 1693                  | 273                       | -1660   | 306               |
| 2009 | 1815                  | 293                       | -1754   | 353               |
| 2010 | 1942                  | 313                       | -1852   | 404               |
| 2011 | 2079                  | 336                       | -1955   | 460               |
| 2012 | 2225                  | 359                       | -2063   | 521               |
| 2013 | 2381                  | 384                       | -2178   | 587               |
| 2014 | 2546                  | 411                       | -2298   | 658               |
| 2015 | 2723                  | 439                       | -2426   | 737               |

Source: Allen Consulting Group calculations.

Table 3.3 shows only the reductions in hospital expenditures resulting from an expansion in the use of pharmaceuticals for cardiovascular disease. The modelling exercise did not extend to the impacts of this innovation on other healthcare costs or macroeconomic variables (primarily workforce participations). For example:

- expanded use of pharmaceuticals for this population is likely to increase participation in the workforce by persons suffering from cardiovascular disease, as well as by their carers and families; and
- there possibly could be indirect increases in healthcare expenditures as the expansion of pharmaceutical therapies for cardiovascular disease allows people to live longer. This of course makes the point that it is social benefit — and not merely quantifiable economic benefit — that matters.

These issues underscore the challenge of quantifying the impact of advances in pharmaceutical innovation on healthcare expenditures and reinforce the need for a comprehensive assessment strategy that considers the range of costs and benefits jointly.

### 3.4 Conclusions

Although increases in pharmaceutical expenditures have attracted a great deal of attention recently, it is misleading to consider the expenditure on pharmaceuticals separately instead of as part of overall resource use in the healthcare system. This chapter has taken a broader view of the costs and benefits of pharmaceuticals. This leads to a more comprehensive appreciation of the costs and benefits of pharmaceuticals, which includes the impact on other forms of healthcare expenditure, and the benefits associated with increases in productivity as well as improvements in general wellbeing and quality of life.



## Chapter 4

# Promoting cost-effective use of pharmaceuticals

### KEY POINTS

Cost-effective use of pharmaceuticals entails significant challenges, not least because there is a significant level of uncertainty (in the aggregate) about their benefits and costs – particularly in the case of new pharmaceutical treatments.

This is the case from the perspectives of both the Australian Government, as the main source of funding for pharmaceuticals, and pharmaceutical companies, as the developer of new pharmaceuticals.

It is a risk management exercise for the Government:

- if the Government spends too much on subsidising pharmaceuticals, it risks over-utilisation, which is not only inefficient but could also lead to poorer health outcomes in some cases;
- if the Government spends too little on pharmaceuticals, it also risks poorer health outcomes from restricting access to the benefits of pharmaceuticals, and also from discouraging the pharmaceutical industry to invest in the research and development of new, more effective medicines.

Australia needs an approach to cost effectiveness that recognises the uncertainties and balances the contrasting objectives of health and industry policy.

Australia has been at the forefront of implementing measures to promote the cost-effective use of pharmaceuticals. It was the first country to introduce a formal and systematic approach to the economic assessment of pharmaceuticals.

It is important to ensure that Australia's approach to promoting cost effectiveness can meet the future challenges facing the public financing of pharmaceuticals, particularly the development of innovative but high-cost therapies for the prevention and treatment of diseases.

The report puts forward for discussion approaches which recognise the importance of dealing with the uncertainties. They are based on risk sharing among the Government, pharmaceutical companies, and healthcare providers, and they acknowledge the challenges and opportunities of the evolving world of pharmaceuticals. They have the potential to help better manage the risk of funding pharmaceuticals in the context of significant uncertainty about both the benefits and costs.

### 4.1 A risk management perspective

Advances in pharmaceuticals, which offer the promise of new and more effective medicines to prevent and treat disease, are dependent on research and development. Cost effectiveness in healthcare requires assessing the benefits of a medical product or service in light of its costs. It is about ensuring 'value for money' or to put it more technically, to be cost effective, a technology should produce a desired health outcome at a lower cost per unit of improvement than other technologies.<sup>56</sup>

There is substantial uncertainty associated with both the benefits and the related costs of pharmaceuticals, particularly from the broader point of view put forward in chapter 3. This is the case for both the Government and the pharmaceutical companies.

<sup>56</sup> Productivity Commission 2004, *Impact of Advances in Medical Technology on Healthcare Expenditure in Australia*, Productivity Commission Issues Paper, September.

From the perspective of the pharmaceutical companies, as noted earlier, innovation is complex and comes at a high cost to developers. Developing, testing, and gaining approval for a new medication is costly, time consuming and risky. On average, it takes 12 to 15 years to develop and test a new medicine. To preserve the incentives for innovation given the risks, it is necessary to allow innovators to not only recover their costs but also to make adequate profits in the cases where discoveries lead to effective treatments. This is offered through the patent system and pricing policies which balance adequate returns on investment with affordable access to essential medicines.<sup>57</sup>

From the Government's perspective, with responsibilities for approving the listing and pricing of pharmaceuticals and as the main source of funding, risk management is a crucial element of its pricing responsibilities. If the Government *spends too much* on pharmaceuticals, it risks adverse events following from too little expenditure on alternative investments in health or other goods and services. It also risks over-utilisation, which is not only inefficient but could also lead to poorer health outcomes. On the other hand, if the Government *spends too little* on pharmaceuticals, it also risks poorer health outcomes from restricting access to the benefits of pharmaceuticals, and also from discouraging the pharmaceutical industry to invest in the research and development of new, more effective medicines.

The aim of a risk management approach to pharmaceutical development, approval and funding is not to eliminate the risks — the risks are an essential element of the research and discovery process that leads to innovative, more effective pharmaceuticals. From the Government's perspective, the aim is to manage the risk on behalf of the community to attain the benefits. One way of doing this is through regulatory processes that encourage pharmaceutical companies to bear the risk of research and development to bring improved products to market, while ensuring the cost to the budget is affordable and sustainable in the long term.

Section 4.2 below outlines the key government measures currently in place to promote the cost-effective use of pharmaceuticals. Section 4.3 puts forward some ideas based on risk sharing which have the potential to help better manage the risk of funding pharmaceuticals in times of a high degree of uncertainty on both the benefits and costs.

## **4.2 Current strategies for cost effectiveness**

### ***Pharmacoeconomic assessment***

Australia was the first country to introduce a formal, organised and systematic use of pharmacoeconomic assessment by government payers when in 1993, the Government required pharmaceutical companies to produce economic data in support of applications for the listing of new pharmaceutical products on the PBS. Pharmacoeconomic assessment is the practice of assessing and comparing both the benefits and costs of a pharmaceutical product, or therapeutic class of products.<sup>58</sup> In Australia, the practice is used to determine which products are purchased under the PBS and at what prices products should be purchased.

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<sup>57</sup> S. Jacobzone 2000, 'Pharmaceutical policies in OECD countries: Reconciling social and industrial goals', *Labour Market and Social Policy – Occasional Papers* No. 40, pp. 17 and 33.

<sup>58</sup> Ibid.

The Pharmaceutical Benefits Advisory Committee (PBAC) is the Government's main mechanism to assess the cost effectiveness of pharmaceuticals. It makes recommendations and gives advice to the Minister about which medications and medicinal preparations should be made available as pharmaceutical benefits.<sup>59</sup> The committee is required to consider the effectiveness and cost of a proposed benefit compared to alternative therapies. Box 4.1 describes the process that is followed for a medication to become listed on the PBS.

Box 4.1

#### HOW A MEDICATION BECOMES LISTED ON THE PBS

The Therapeutic Goods Administration (TGA) must register new pharmaceutical entities before they are marketed in Australia. Registration is based on an assessment of quality, safety and efficacy, a process that often involves the Australian Drug Evaluation Committee (ADEC).

If ADEC recommends that the medication should be available for sale, then a sponsor (usually the pharmaceutical company) can apply to the Pharmaceutical Benefits Advisory Committee (PBAC) for listing on the PBS.

The PBAC assesses the medication's effectiveness, including its cost effectiveness, and advises the Minister for Health and Ageing if the medication should be listed on the PBS. It may:

- recommend a medication for subsidy listing as acceptably cost effective at the requested price or at a lower than requested price; or
- recommend a medication for subsidy listing with tighter restrictions than those proposed in the submission (this is known as targeting); or
- reject a medication for subsidy listing on clinical and/or cost effectiveness grounds.

If the Minister accepts the recommendation of the PBAC, the medication is then referred to the Pharmaceutical Benefits Pricing Authority (PBPA), which negotiates with the pharmaceutical manufacturer on the price at which the medication will be listed on the PBS and advises the Minister accordingly.

Source: A. Biggs 2003, *The Pharmaceutical Benefits Scheme – an Overview*, Parliamentary Library E-Brief, Parliament of Australia, [www.aph.gov.au/library/intguide/SP/pbs.htm](http://www.aph.gov.au/library/intguide/SP/pbs.htm), accessed 9 February 2004.

#### *Influencing prescribers*

Given the importance of prescriber behaviour in the use of pharmaceuticals, the Government has put in place several measures to influence clinicians' prescribing as a way to enhance the effectiveness of pharmacoeconomic assessment by the PBAC. The rationale behind these measures is a perception that inappropriate prescribing is widespread. One example of inappropriate prescribing is referred to as 'leakage', whereby relatively expensive medications are prescribed with a PBS subsidy for conditions for which the medications are not subsidised under PBS restrictions due to the availability of a more cost-effective alternative.

<sup>59</sup> Information on the PBAC is available from the PBAC web page, at <http://www.health.gov.au/internet/wcms/publishing.nsf/Content/health-pbs-general-listing-committee.htm>.

Although some have concluded that inappropriate prescribing is responsible for a major proportion of unnecessary PBS cost increases,<sup>60</sup> there is little data or evidence of its overall incidence. Estimates of the annual cost to the Commonwealth budget of leakage alone have ranged from \$50 million to \$1 billion in 2000–01.<sup>61</sup>

The Government uses several approaches to influence the way in which clinicians prescribe medicines, including:

- the development and dissemination of prescription guidelines;
- the use of restricted listing for medications on the PBS. The last decade has seen a pronounced increase in the number of items that have been listed on the PBS with restricted or authority required benefits. Most items listed on the PBS are now in the categories of a restricted or authority required benefit; and
- a range of activities to encourage clinicians to prescribe within the PBS guidelines, including audit and feedback involving the Health Insurance Commission (HIC), as well as various education campaigns.

### ***Influencing consumers***

To enhance the effectiveness of pharmacoeconomic assessments, the Australian Government also seeks to influence consumers' use of pharmaceuticals. This is done particularly through the following pricing policies designed to encourage consumers to choose the most cost-effective medications:

- the brand premium policy, under which the PBS price is set with reference to the generic product when the patent for the underlying preparation of a medication has expired. If a patient is prescribed a brand which is not the lowest priced, they will be required to pay the brand premium, which is the price difference between the lowest priced brand and the brand prescribed;
- the brand substitution policy, under which a pharmacist can substitute an equivalent brand other than that prescribed by the patient's doctor without reference back to the prescriber; and
- the therapeutic group premium, under which prices paid by the PBS for medications in four therapeutic groups are set at the lowest priced medication within that group of (not necessarily bio-equivalent) medications. The patient pays the price difference (the therapeutic group premium) if a more expensive medication within the group is prescribed.

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<sup>60</sup> K. Harvey 2002, *Securing the future of the Pharmaceutical Benefits Scheme?*, The Drawing Board: Australian Review of Public Affairs, 3 June, [www.econ.usyd.edu.au/drawingboard/digest/0206/harvey.html](http://www.econ.usyd.edu.au/drawingboard/digest/0206/harvey.html), accessed 18 February 2004.

<sup>61</sup> M. Rickard 2002, *The Pharmaceutical Benefits Scheme: options for cost control*, Parliamentary Library Current Issues Brief no. 12 2001-02, Parliament of Australia, [www.aph.gov.au/library/pubs/cib/2001-02/2cib12.htm](http://www.aph.gov.au/library/pubs/cib/2001-02/2cib12.htm), Accessed 9 February 2004, p. 6.

In particular, the brand premium and substitution policies have had a significant impact, with now the majority of prescriptions dispensed at the benchmark level.<sup>62</sup> The Australian experience supports the OECD's view that, on the whole, generics appear to have only developed where strong financial incentives that impacted patients, pharmacists and/or prescribing physicians had been implemented. This involves substitution rights for pharmacists and incentives for patients to buy generic medications.<sup>63</sup> Although the development of generics is a very important mechanism to promote cost effectiveness, it is nevertheless important that governments balance the desire to disseminate important new medications as quickly as possible at as low a price as possible with the need to allow higher prices for those still on patent in the interests of preserving the long-term incentives for important innovations.<sup>64</sup>

### ***Extensions to current policies***

Suggestions on ways to improve the Government's policies to promote the cost-effective use of pharmaceuticals have tended to focus on extending the current approaches, for example:

- *a system of periodic reviews of the cost effectiveness of listed medications.* Currently there is no ongoing system of regular reviews of the PBPA's initial cost effectiveness assessment of the prices of listed medications. It may be that some medications are less cost effective now than they were early in release (for example, given advances in medical technology), or alternatively, than some medications are more cost effective (for example, as a result of post-marketing surveillance and additional data becoming available);
- *increased application of price-volume agreements.* Currently, the PBPA sometimes negotiates the prices it pays for medications partly on the basis of the anticipated utilisation of those medications. This is particularly true where unit prices are reasonably high and there is the potential for significant volumes, or where there is uncertainty about future volumes. Such arrangements have also been negotiated where there is potential for volumes to increase significantly due to use outside PBS restrictions. Price-volume agreements enable a more accurate matching between the actual cost effectiveness of a listed medication and the price paid for it;<sup>65</sup>
- *greater encouragement of the prescription of generic medications;* and
- *an extension of the assessment for cost effectiveness to medications currently on the PBS that were listed pre-1993.*

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<sup>62</sup> Pharmaceutical Benefits Pricing Authority 2003, *Annual Report 2002-2003*, Canberra, p. 8.

<sup>63</sup> Jacobzone 2000, *op. cit.*, p. 42.

<sup>64</sup> *Ibid.*, p. 43.

<sup>65</sup> Rickard 2002, *op. cit.*, pp. 16-7.

### 4.3 Risk management strategies

Although Australia has been at the forefront of innovative policies to promote the cost-effective use of pharmaceuticals, it is important to ensure that Australia's approach can meet the challenges facing the public financing of pharmaceuticals, particularly the changing world of pharmaceutical development and usage. As discussed in section 2.3, the development of pharmacogenomics is likely to have a substantial influence on the development of new pharmaceuticals, with major implications for the approval, listing and funding of pharmaceuticals.

The most likely impact of research in pharmacogenomics in the medium term is the development of genetic tests that determine in advance which drugs will work for a given individual and which will not. Thus, this field has the potential to play an important role in improving safety, efficacy and cost effectiveness. Adverse reactions to medicines have significant costs, both in human and monetary terms. Moreover, considerable resources are wasted on prescribing medicines that have little or no effect in particular patients.<sup>66</sup>

The predicted developments from pharmacogenomics offer both challenges and opportunities for the Australian healthcare system.

In terms of *challenges*, the PBS is, and will continue to be, under increasing pressure to fund new innovative, targeted and high-cost therapies for the prevention and treatment of previously unmanageable diseases. Developments in pharmacogenomics may represent major advances in prevention and treatment, and potentially have a great impact on health outcomes, but they often come at high costs. Unlike medications that are applied equally across individuals with a particular condition, targeted medications will need to recover research and development costs from a smaller population of users.<sup>67</sup>

Consistent with this, Professor Lloyd Sansom, Chairman of the Pharmaceutical Benefits Advisory Committee, has recently suggested that:

the greatest challenge to the PBS is the availability of agents resulting from molecular design and the human genome project. We are already seeing the marketing of such drugs (e.g. Imatinib, Etanercept) at a cost which is generally much higher than we have previously seen for new agents. Further, the benefit of many of the newer anticancer drugs will be incremental resulting in extremely high and unfavourable cost effectiveness ratios, yet the community (and health professional) demand for these agents to be subsidised continues to grow.<sup>68</sup>

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<sup>66</sup> Nuffield Council on Bioethics 2003, *Pharmacogenetics: ethical issues*.

<sup>67</sup> L. Noah 2002, 'The coming pharmacogenomics revolution: Tailoring drugs to fit patients' genetic profiles', *Jurimetrics*, 43, 1-28.

<sup>68</sup> Quoted in L. Brown, A. Walker, A-M. Waters, A. Harding and L. Thurecht 2002, *Funding of High Cost Biotechnology and Other Innovative Targeted Therapies under the Pharmaceutical Benefits Scheme*, NATSEM Position Paper, at [www.natsem.canberra.edu.au](http://www.natsem.canberra.edu.au), p. 9.

In terms of *opportunities*, a key feature that differentiates these new interventions from the traditional small molecules currently being listed on the PBS is that these high-cost macromolecule solutions are ‘targeted’ therapies, that is, they are designed for use in a well defined targeted group of patients who have specific biological markers.<sup>69</sup> It is this target group of patients who will respond and benefit most from therapy. Thus, it should be possible to establish clear criteria for patient eligibility for the new targeted therapies to optimise health outcomes, accurately predict the budgetary implications to government if listed on the PBS, and minimise opportunity for leakage.

Developments in pharmacogenomics also have the potential to optimise the funding of pharmaceuticals. As noted previously, discoveries in this field will reduce the number of adverse medical events — each of which is typically associated with substantial healthcare expenditures and loss of general wellbeing. Moreover, pharmacogenomics will allow researchers to better target the most promising new medicines for costly clinical trials.

This section puts forward for discussion approaches based on risk sharing among the Government, pharmaceutical companies and healthcare providers, which acknowledge the challenges and opportunities of the evolving world of pharmaceuticals. They have the potential to help better manage the risk of funding pharmaceuticals in times of significant uncertainty about both the benefits and the costs. Moreover, because these risk-sharing models rely on the presence of high-quality data describing the health outcomes associated with pharmaceuticals and other forms of healthcare, their use will put a premium on the collection of such information — which will also benefit analyses of the cost-effective use of pharmaceuticals generally.

### ***Risk-sharing approaches***

#### ***UK funding models based on outcome guarantees***

A funding model that has emerged in the UK in response to the development of high-cost biotechnology medicines and other targeted therapies is risk sharing based on outcome guarantees. The model begins with acknowledging that it is necessary to follow the pharmaceutical company's recommendations for use to obtain maximum health benefits from a medicine. However, variations in clinical prescribing practice and patient compliance mean that medicines are not always used under optimum conditions.

One mechanism of achieving maximum benefit is to set up an ‘outcomes guarantee’, in which a pharmaceutical company and prescribing stakeholders agree on the outcomes that they would expect from a medication in a given indication. If the medicine fails to fulfil expectations, the pharmaceutical company refunds the health service for the cost of the medicine. This encourages the pharmaceutical company to promote responsible prescribing and ensures that healthcare resources are not wasted on ineffective treatments.<sup>70</sup> Examples of outcome guarantees in the UK are outlined in box 4.3 below.

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<sup>69</sup> Brown et al 2002, op. cit., p. 2.

<sup>70</sup> S. Chapman, E. Reeve, G. Rajaratnam and R. Neary 2003, ‘Setting up an outcomes guarantee for pharmaceuticals: new approach to risk sharing in primary care’, *British Medical Journal*, Vol. 326, 29 March, p. 707.



Box 4.3

**EXAMPLES OF OUTCOMES GUARANTEES FOR PHARMACEUTICALS****Treatment of high blood cholesterol**

In 1999, North Staffordshire Health Authority, Parke-Davis (now Pfizer), and Keele University entered a pilot collaboration to provide an outcomes guarantee for statins in lowering blood cholesterol concentrations. The outcomes guarantee was aimed at protecting the health service from paying for a medicine if it did not work, for example, because it was inappropriately prescribed or was not as effective as claimed.

**Treatment of multiple sclerosis**

Recently the UK Department of Health agreed to a 'groundbreaking' scheme for funding expensive medicines for the treatment of multiple sclerosis (MS). Under a ten-year agreement, the manufacturers of the medicines will only be paid in full if the treatment 'lives up to its promise'; that is, if treatment trials in individual patients show the medications are effective.

Source: S. Chapman, E. Reeve, G. Rajaratnam and R. Neary 2003, 'Setting up an outcomes guarantee for pharmaceuticals: new approach to risk sharing in primary care', *British Medical Journal*, Vol. 326, 29 March, pp. 707-9; S. Mayor 2001, 'Health Department to fund interferon beta despite institute's ruling', *British Medical Journal*, 10 November, p. 1087; D. Taylor 2001, 'Funding medicines for people with multiple sclerosis', *British Medical Journal*, 15 December, pp. 1379-80.

It is too soon to say whether outcome guarantees will be successful. There are, for example, some concerns with the UK government's risk-sharing scheme for multiple sclerosis drugs, which has faced many implementation challenges.<sup>71</sup> However, an appropriately framed strategy could help resolve uncertainties about cutting-edge treatments, where the ultimate impact on health outcomes of costly medications is not known throughout most of the life of their patents, or of the patients taking them.<sup>72</sup> In addition, there would be other benefits — for example, there may be faster listing of high-cost medicines countered with rigorous monitoring processes of what happens post-listing. This could be a new approach, with the possibility of altering decisions going forward based on what happens to medicines in the field by monitoring real outcomes in real life.

***An Australian risk-sharing scheme***

The recent listing of etanercept for the treatment of rheumatoid arthritis under Australia's PBS provides another example of a risk-sharing approach to the funding of pharmaceuticals.

The approach emerged from a unique collaboration between the key stakeholders — the PBAC, the pharmaceutical company (Wyeth), rheumatologists and consumers (Arthritis Foundation of Australia and Arthritis Research Task Force).<sup>73</sup> The stakeholders agreed on eligibility criteria for the initial prescription and for continuation of treatment beyond three months. Prescribing rights were limited to rheumatologists and evidence was required that patients agreed to abide by a decision to stop treatment at three months if response criteria were not met. Under these conditions, Wyeth believed that expenditure under the PBS would not exceed \$100 million a year and agreed to pay for any expenditure above this figure.

<sup>71</sup> M. Mrazek and E. Mossialos 2004, 'Regulating pharmaceutical prices in the European Union', in E. Mossialos, M. Mrazek and T. Walley (eds), *Regulating Pharmaceuticals in Europe: Striving for Efficiency, Equity and Quality*, Open University Press, Berkshire, p. 120.

<sup>72</sup> D. Taylor 2001, 'Funding medicines for people with multiple sclerosis', *British Medical Journal*, 15 December, p. 1380.

<sup>73</sup> C, Lu et al, 2004, 'Access to high cost drugs in Australia: Risk sharing scheme may set a new paradigm', *British Medical Journal*, 329, 415-6.



This risk sharing agreement provides incentives to the pharmaceutical company to promote the pharmaceutical in accordance with the restrictions, and to prescribers and consumer organisations to avoid leakage to individuals outside the restrictions. An evaluation of the agreement will require accurate and timely data on both prescription rates (available from the HIC) and health outcomes for individual patients. The Australian Rheumatology Association has recently established a national database to track patient outcomes over the long term, which will allow an assessment of whether the restrictions are achieving the desired clinical responses.<sup>74</sup>

Again, it is too early to say whether this approach will be successful. However, the lessons learned from this and other risk sharing approaches to the funding of pharmaceuticals will assist in the development of ways to ensure the sustainability of publicly funded access to effective but expensive treatments.

#### **4.4 Conclusions**

Although Australia has been at the forefront of innovative policies to promote the cost-effective use of pharmaceuticals, it is important to ensure that its approach can meet the challenges facing the public financing of pharmaceuticals, particularly the changing world of pharmaceutical development and usage. Many of these challenges arise because of the significant level of uncertainty about the benefits and the costs of pharmaceuticals — particularly in the case of new and often high-cost pharmaceutical treatments.

This chapter has underscored the need for Australia to adopt an approach to cost effectiveness that recognises the uncertainties and balances the contrasting objectives of health and industry policy. It put forward proposals that are based on risk sharing between the Government and pharmaceutical companies, acknowledging the challenges and opportunities of the developing pharmaceutical field. These proposals have the potential to help better manage the risk of funding pharmaceuticals in the context of significant uncertainty about the magnitude of both the benefits and the costs.

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<sup>74</sup> Ibid.