

The Quality Use of Medicines: Serving Health and Economic Objectives?

**Background Paper
National Medicines Policy Partnerships
Workshop**

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October 2003

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The purpose of this paper is to provide a perspective on the issues to be discussed at the National Medicines Policy Partnerships Workshop, and to stimulate, but not to intrude into, that discussion.

My theme is that there is an urgent need, in the interests of better health outcomes for the Australian people, to transcend the current environment of crisis and conflict linked to cost containment in medicines policy. One key to this is a real focus on the quality use of medicines (QUM) – ensuring that all Australians get all of those cost effective medicines, and only those medicines, necessary for the best available health outcomes. Such a focus is in the true interests of all parties, and can serve both health and economic objectives, especially given the changes that are taking place in health and medicines world wide.

Serious attention to the use of medicines is, I argue, the forgotten dimension of the technological revolution in health. However, many aspects of emerging trends reinforce the need for greatly enhanced focus on QUM. The age of the big 'blockbuster' is waning, as medical research breaks diseases such as cancer or asthma down into a multitude of more specific conditions, and powerful new discovery and testing technologies lead to an increase in new targeted medicines. The result will be a proliferation of old and new medicines on the market, many with quite narrowly defined applications. The health benefits of this situation can only be maximised, and the risks minimised, by effective partnerships in the quality use of medicines.

Two factors standing in the way of real focus on QUM are the intense emphasis on short-run cost containment at the Federal level and the low and declining prices for innovative medicines in the PBS. Addressing these contextual factors is an important part of generating real partnerships, among all stakeholders, to advance QUM. Four specific areas in which the stakeholders may be able to work together immediately to advance QUM are discussed briefly. These are the development of integrated data bases and information systems to document health outcomes and to monitor the use of medicines; ways of introducing commercial incentives into pricing arrangements to encourage QUM; clearer definition of the roles of all parties in the national QUM strategy and the development of public policies to achieve the various goals of QUM.

¹ Director of the Centre for Strategic Economic Studies at Victoria University, Melbourne. The author is grateful to many individuals – in the participating companies and agencies and at the Centre – for comments on earlier drafts of this paper. It remains, however, entirely his own responsibility, and does not necessarily reflect the views of any other agency, company or individual. Preparation of the paper was supported by funding from The Pharmaceutical Alliance (TPA), a collaboration of Eli Lilly, GlaxoSmithKline and Merck Sharp and Dohme, and draws on extensive research undertaken on related issues at the Centre over the past two years.

In developing this theme, the paper sets out to do four things. The first is to place the immediate issues of the quality use of medicines in a broader international context (Section 1) and also in a broader Australian context (Section 2). Given the need to review complex material quickly, this is done by advancing and commenting on fourteen propositions seen as relevant to the issues. The second is discuss the neglected issues concerning the use of medicines, and to lay out the various aspects of QUM and the importance of these matters (Section 3). The third is to analyse briefly the basis for, and the obstacles to, serious cooperation in relation to QUM between the parties represented at this workshop (Section 4). Finally, I comment a little on some potential areas of cooperation, having regard to these various contexts, before providing my overall conclusions.

1. Some International Perspectives

1. ***The Centrality of Health Outcomes.*** Many countries have experienced a substantial improvement in health outcomes over recent decades. By international standards, Australia has generally achieved good, cost effective and evenly distributed health outcomes, other than for its indigenous peoples. But much more needs to be done, and serious inequities remain. Further progress can be achieved in part by better use of existing and new medicines.

Effective innovation in health care – whether in medicines, devices, procedures or in public health processes – continues to produce major improvements in human welfare. For both men and women, age-standardised death rates have fallen rapidly in Australia over the past two decades. This improvement has been particularly striking for men, for whom age standardised death rates fell by 35.7% between 1981 and 2000, nearly as much as they did in the six decades from 1921 to 1981 (AIHW 2002).

It is worth recalling that, among developed countries, the Australian health system delivers better than average outcomes at about average costs. In 1997, age standardised death rates for men were lower in Australia than in all OECD countries other than Japan, while for women they were lower than in all OECD countries other than Japan and France (AIHW 2002). Total Australian health expenditure, even at 9.3% of GDP in 2001-02, is still below the average of the major countries.

But we all know that there is very much more to be done, in many areas: encouraging healthy lifestyles, prevention, the misuse of some medicines and the under-utilisation of others, to name but a few. Improving health outcomes for the community as a whole must remain the central objective of health policy, and a central theme of this paper is that important gains can be made by much more systematic attention to the quality use of medicines.

2. ***Innovation in Health Technologies, and the Shift from Services to Technology in Health.*** Driven by the results of massive global investment in medical research, by both the public and private sectors, the health sector is in the midst of an important shift from services to technology, embodied in medicines, equipment and procedures. This 'rotation from services to technology' will continue to be a

dominant factor for the health sector, for biomedicine has replaced information technology as the central focus of global R&D and technological change.

The health system is in the midst of a long term and fundamental shift in balance from services to technologies, from personal services provided by doctors, nurses and hospitals to technology embodied in medicines, equipment and procedures. This increasing use of technology is for each of prevention, treatment and cure, and is again evident in most Western countries.

While cost factors have also played an important role, for example in reducing the length of hospital stays, lying behind this rotation is the fundamental revolution that is taking place in biomedical research and biotechnology. Medical research has been growing rapidly for two decades, and the fruits of that research have become increasingly evident in health systems and in the flow of new products. For example, the annual number of patents for new medicines approved by the US Patent Office in recent years has been about four times the average number approved in the first half of the 1980s.

However, this revolution has a long way to run, and further major impacts will be felt as genomics, proteomics and other emerging sciences lead to new applications. Indeed, biomedicine has in the past five years replaced information technology as the central focus of global technological change. Over the medium term biotech and information technology will increasingly converge at the nano scale, opening up radical new medical options. The new products that emerge will further accentuate the shift from services to technology in health.

3. ***Massive Value of Gains from Improved Health – the Value of Medicines.*** The welfare gains that have been achieved over the past century through improved health outcomes are massive, perhaps equal to all other welfare gains from economic development over this period. The value of medicines lies in their continuing contribution to these broadly based welfare gains.

Much of our contemporary national discussion is about economic matters, such as growth and interest rates. But the point of economic growth is the improvement in human welfare that it delivers. Recent estimates suggest that, of the welfare gains to the human community from economic development over the past century, fully half came from improved health outcomes (Nordhaus 1999). That is, improved welfare from better health is as important as all the other benefits of development put together.

This broader perspective is important in considering the value of existing and new medicines. While only one of several factors generating these welfare gains, medicines have played a central role, and this role will probably be accentuated in the future. The broad welfare perspective on the value of medicines is relevant not only for debates about pricing but also for assessing the social gains to be made through the quality use of medicines.

4. ***The Move to Many Smaller, More Targeted Medicines.*** In recent decades, the pharmaceutical industry has largely relied on a relatively small number of 'one-size-fits-all' medicines with very large markets, and which have been very costly to discover. Technology, market and consumer trends are pushing it towards a larger

number of medicines with smaller markets. These medicines will have shorter development times and be targeted more directly to sharply defined diseases and to individual needs.

There has been much discussion of a decline in 'big pharma' R&D, as the massive resources devoted to R&D by the big companies has led to a declining number of annual approvals of new 'blockbuster' medicines. These medicines - 'one-size-fits-all' medicines with mass markets - have constituted the industry's central strategy for over a decade. In 1992 there were only four blockbusters on the US market; by 2001 there were fifty, and over that period the blockbuster market share had risen from 6% to 45%. But a Datamonitor report in 2002 noted that, while there were four blockbusters in the pipeline for that year, there were only 14 in the system for the six years 2003-08.

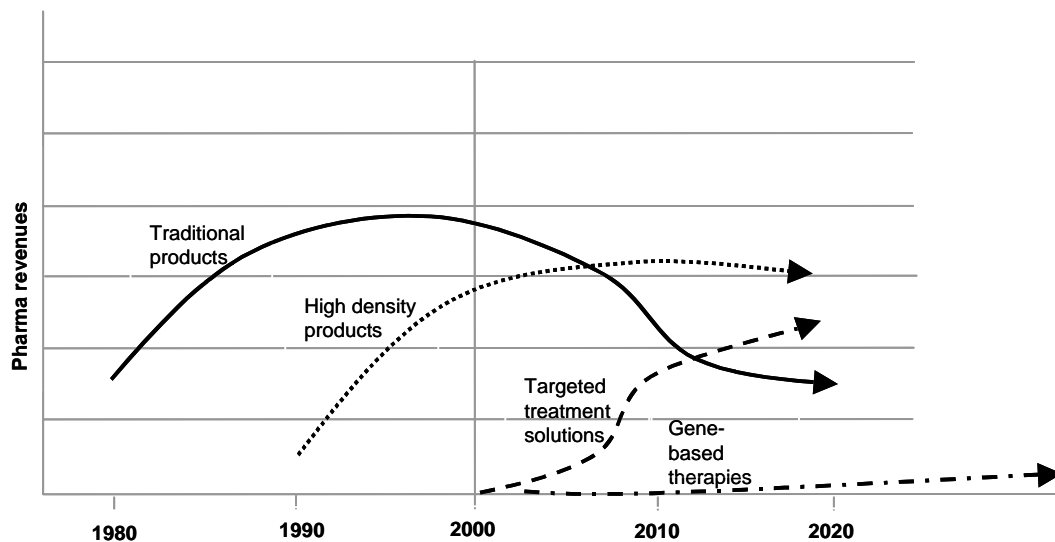
However, this must be seen as the waning of a dominant strategy rather than the beginning of a period in which a smaller number of new medicines coming on to the market. The important recent report *Pharma 2010: The Threshold of Innovation* (IBMCS 2002) identifies three main types of drug products playing a key role in the future market. The first of these are called high density medicines, which are typically secondary treatment medicines, especially in oncology, marketed primarily to or through specialists.

The more significant trend is seen as the development of targeted solutions, where companies develop overall treatment solutions (diagnostics, medicines, monitoring and review systems and educational packages) to treat very specifically defined disease states. These solution packages, an early stage of the widely heralded personalised medicine, will be based on much improved knowledge of specific diseases and of the pathways and responses of different types of individuals to particular medicines. They will be developed to market through a much more interactive regulatory process, and will involve much closer monitoring of use and outcomes. Such a more interactive process - involving a much more continuous process of data flow, feedback, staged approval and monitoring of medicines after approval - is already firmly on the agenda of the FDA in Washington. It could reduce both approval times and development costs significantly.

In the longer term, progress in genomics and proteomics is likely to see gene-based preventative and therapeutic programs put in place, targeting medicines and other treatments to the specific genetic characteristics of the individual. Chart 1 shows the expectations of the authors of the report cited above for the time profile of these four medicine types in the US market. Perhaps the most striking features of that chart are the early decline in the share of blockbusters and the projected rise over the next 5-10 years of targeted solutions.

These new medicines are likely to be biologicals, and to have the advantage over chemical molecules of being more readily accepted by the human body. While targeted at more specific markets than the blockbusters, they will be less costly to develop over a shorter time frame, thanks to advanced computing methods and more interactive regulatory processes.

Chart 1. The Emergence of Different Product Types



Source: IBMGS (2002)

5. *The Forgotten Dimension – Increased Imperatives for the Quality Use of Medicines.* These trends will challenge existing regulatory processes and necessitate closer interaction between consumers, medical professionals, government authorities and drug companies. They will enforce closer attention to the quality use of medicines.

The main focus in medicines over the past decade or more has been the development, supply, safety and pricing of a wide array of new medicines, many with wide application. But the forgotten dimension in all the rush has been the use of those medicines. In almost every country, the focus of public policy has been on the testing of new medicines for safety and efficacy, largely through clinical trials, and the pricing of those medicines. Given constraints emerging from these processes, the use of these new medicines has been determined by the interaction of market forces and medical practices. But there is little knowledge in any country of how these medicines are used in practice - who gets them and who fails to get them, how effective they are, how much waste and duplication is involved, how extensive are the adverse effects of the use of medicines, and so on. Trends in overall health outcomes indicate that the welfare effects of these new medicines have been strongly positive. But the indications, reviewed briefly in Section 3 below, are that costs associated with the inadequate use of medicines are massive.

Looking 3-5 years out, drug markets are likely to be much more complex than they are today. While the medicines approved over the past 10-15 years will still be in the market, as will the new medicines currently at or near the approval stage, many new high density medicines will be becoming available, as will the first of the new generation of targeted biologicals. This is a potential nightmare for regulated and highly subsidised systems trying to manage costs and to achieve both the cost-effective use of medicines for specific indications and also to ensure that everyone in the community gets, and uses properly, the best medicines for them.

These trends will raise the stakes, already high, for the quality use of medicines. The forgotten issue of today will become much more acute in the future, and it seems inevitable that health systems around the world will be forced to address it. This may pose special challenges in highly segmented systems, such as Australia, where the value and use of medicines must be considered in isolation from broader health and economic impacts.

6. ***Patent Expiries and the Increasing Importance of Generics Policy.*** At the same time, as the initial 'blockbuster' stage of the medicines revolution matures, a surge in patent expiries is in prospect and an increased proportion of needs will be met by medicines that are out of patent. How systems handle out-of-patent molecules will be very important, in terms of costs, the return to innovation and the quality use of medicines.

One consequence of the surge in blockbusters over the past fifteen years is that the industry is currently entering a surge in patent expiries on those blockbusters. One source reports that between 2002 and 2007 patents in the US will expire on 35 medicines with market sales of US\$73 billion, or about one third of the US market (IBMGS 2002). Although patent expiry dates differ across countries, similar trends are likely in Australia.

For some time the value of patents has been eroded by the competitive pressure from patented medicines with different molecular structure but similar function, and by indirect pressure from generic versions of such competing molecules, when they come out of patent. Nevertheless, the price of an innovative drug falls significantly when it goes out of patent, and different systems use different methods to capture the value of that fall in cost for the consumer or the taxpayer. In the US, for example, prices for medicines covered by patents are high, but on patent expiry generics enter the market at 15-20% of the price of the innovative drug and quickly capture the bulk of the market. In some other systems, prices for medicines that are within patent are low and the entry of generics is controlled, so that there is little impact on total costs when patents expire.

In my view, it is important that this value of patent expiries is captured in a way that preserves a reward for innovation in the drug pricing system and which is conducive to increased rather than reduced focus on QUM.

7. ***The Revealing Analogy with the Information Technology and Telecommunications (ITC) Industries.*** The analogy with the information technology and telecommunications industries, which have been transformed several times over in the past two decades, is useful in thinking about the continuing revolution in medicines, and its implications for Australia.

The ITC industries, and the technological advances that drove them, were the central drivers of change in the global economy in the 1980s and the 1990s. Given that this role is now being taken by the biomedical and related industries, albeit on the back of enormous computing and communications capabilities, it is instructive to use the ITC case as a lens through which to view current and prospective trends in medicines.

There are many parallels, but of course also many differences, between the two groups of industries. But issues concerning health and medicines are so vast and so absorbing that much discussion of them takes place in a vacuum, with little perspective on broader changes in technology and or in other industries. Using the information industries as a lens or a mirror helps to see these issues in a new light.

For example, in thinking about how policy might deal with the explosion of technological change in medicines, it may be useful to keep the analogy with information technology in mind. In my view, such analogy serves to highlight the intense challenges closely regulated systems face as drug technologies change, and the need for very serious consideration about how such systems should adapt if they are to survive and continue to deliver important social benefits. The information technology analogy is also useful in highlighting the rapid change in corporate structure that such episodes bring, and also the fact that technologies, as they mature and become expressed in commodities (generics in the pharma case), also become cheaper. The latter fact is widely ignored in projections, such as in the *Intergenerational Report* in Australia and the *Wanless Report* in the UK, of the future cost of medicines.

2. Some Australian Perspectives

There are many aspects of the Australian situation that could be highlighted as relevant to our present topic, especially given this changing global context, but I confine myself below to five key points.

8. ***Health as a Growth Industry – Issues about Government Costs and Efficiency of Resource Use.*** Like the information industries, health is a growth industry in Australia and elsewhere. In general it is good to have growth industries, which are expanding through meeting genuine human need and consumer demand. Concerns about the growing share of GDP going to health seem to relate to the high level of dependence on government funding and to doubts about the efficiency of resource use.

There is much discussion about the level of national spending on health. Is it too high? What is an appropriate level of spending? How can it be contained? What issues are raised by rapid growth in spending on pharmaceuticals?

It is hard to come to grips with these partly empirical, partly normative issues. The analogy with IT may help us to think more clearly. Table 1 shows national expenditure on information technology and on health for 1992-93 and 2001-02. Spending on IT is still a little below that on health, but is now comparable (8.6% of GDP compared to 9.3%), but it has grown more rapidly over the past decade. Is IT spending, or health spending, too high or too low? In public debate a low IT share is generally seen as bad (we are missing out on the benefits of new technology) while a high health share is bad (we are wasting money on health). What do these questions and judgements mean?

Table 1. Expenditure on information technology and health

	1992-93		2001-02	
	\$ billion	Share of GDP	\$ billion	Share of GDP
Information technology	24.2	5.7	61.5	8.6
Health	35.1	8.2	66.6	9.3

Source: IT data, Houghton (2003); Health data, AIHW (2003).

In principle, a rapidly growing economic sector (rising share of GDP) is a good thing if growing expenditure and output contributes positively to human welfare. Can one argue that recent, and perhaps future, growth in health expenditure has generated increased welfare, so that it should be welcomed as a growth industry, like IT?

These considerations bring out one key difference with health: the dominant role of government. The public purse meets over 70% of health expenditure, by comparison with a much smaller percentage for national IT. The social importance of health is the rationale for this, but is not the social divide in access to and use of IT goods and services also of great importance?

These considerations point in another direction: that perhaps the major problem with increasing health expenditure is the cost it imposes on government, and on the Commonwealth Government in particular, rather than on the community as a whole.

Table 2. Health expenditure, by sector, 1991-92 to 2000-01

	Levels		Percent change	Shares	
	1990-91	2000-01	1990-91 to 2000-01	1990-91	2000-01
	(\$ billion)		(%)	(%)	
Commonwealth	13.2	28.8	118.2	42.2	47.4
State and Local	8.0	13.7	71.3	25.6	22.5
Non-government	10.1	18.3	81.2	32.3	30.1
Total	31.3	60.8	94.2	100.0	100.0

Source: AIHW (2002).

Table 2 shows that the Commonwealth share of rising national health spending was itself rising during the 1990s. In terms of shares it rose from 42.2% to 47.4%, and that of the States and of the non-government sector fell. Indeed, all the growth in health costs over the 1990s, in terms of the share of GDP, was met by the Commonwealth. This inevitably means that many health decisions are, in the end, not individual or welfare decisions but political and financial ones.

9. **The Continuing Shift in Health Spending from Medical and Hospital Services to Goods.** Reflecting the rotation from services to technology and other factors, there has been a sharp shift in the share of total recurrent health spending in Australia from hospital and medical services to medicines, aids and appliances over the past decade. This shift is likely to continue.

One way of seeing this, which also foreshadows other issues to be touched on below, is to look at the changing composition of total recurrent health expenditure (Table 3). Over the 1990s there was a 5.7 percentage point decline in the share of expenditure going medical services and hospitals, with a corresponding increase in the share going to medicines, aids and appliances. Similar trends are apparent in the USA and other countries.

Table 3. Shares of recurrent health expenditure, 1991-92 and 2000-01

	Share, 1991-92 (%)	Share, 2000-01 (%)	Change in share (percentage points)
Medical services	19.0	18.0	-1.0
Hospitals	39.7	35.0	-4.7
Public hospitals	32.5	27.6	-4.9
Private hospitals	7.1	7.5	0.4
Medicines, aids and appliances	12.2	17.9	5.7
Other	29.1	29.1	0.0
Total	100	100	..

Source: AIHW (2003).

Are more and better medicines reducing the demand for hospitalisation? Or is rising spending on technology squeezing hospital spending? While this paper is not the place to discuss this important issue in any detail, the most plausible answer is that both factors are at work. But the magnitude of the shift over a decade is such that it must, to a significant degree, reflect strong structural changes in the health sector, and particularly the rotation from services to technology. Recent work in the US (eg Lichtenberg etc) has recently identified strong impacts of new drug use in terms of reduced hospitalisation rates and health system costs. This highlights again how misleading it is to focus on the management of medicines as a single budget line, without considering broader linkages through the health system.

10. **Severe Segmentation in Australian Health.** The Australian health system is badly affected by the many forms of segmentation – Federal and State, public and private status, medicines and services, urban and rural – that are endemic within it. This means that the focus is often on management of the costs and benefits within the segments, rather than on the needs of the individual. Such segmentation gets in the way of the quality use of medicines, but is becoming less viable as the health sector becomes more open, dynamic and informed.

Many argue that health arrangements are heavily distorted by the fragmentation of the current health care system into various segments, such as private versus public hospitals, Medicare versus private health insurers, admitted versus non-admitted patient status, medicines and medical services, Commonwealth and States, and so on. The result is that a number of barriers have arisen preventing patients from accessing affordable and timely health care in ways, or at locations, that are convenient to them.

That fragmentation of health care also leads to fractured perspectives – the silo mentality – resulting in lack of coordination across different segments. The focus is not on the patient but on boundaries between these segments, resulting in avoidable neglect of patients while administrators are chasing false economies for their respective segments – creating higher overall costs for the community. This segmentation seems to be a substantial problem in the health system and a barrier to determining and implementing the most cost effective treatment. This is particularly acute in relation to pharmaceuticals, which are often seen as an isolated cost and not in the perspective of overall societal health benefits.

11. ***Some Key Characteristics of the PBS.*** By international standards, current PBS outcomes are characterised by low and falling prices for innovative medicines; uncertainty about listing timeframes and conditions, and about future prices; and by low use of generics at relatively high prices.

The National Medicines Policy Partnerships meeting is not a discussion about the PBS, but the varying views of many of the parties about that scheme, and how it is currently operating, will be an important sub-text to the discussion. As a consequence, I here note briefly three features of the operations of the PBS that seem relevant to the QUM discussion, but will provide justification here for only one of the elements of Proposition 11, that concerning the falling trend in drug prices.

Measuring comparative drug prices at an aggregate level across different countries, or comparative price levels at different times within a given country, is an extremely complex matter. There are a vast array of medicines, sold in various strengths, packages and formulations, and the formularies used in different countries vary significantly. A given drug prescription (an item) may vary from the very simple to the extremely complicated, and hence be hard to compare in 'quantity' terms.

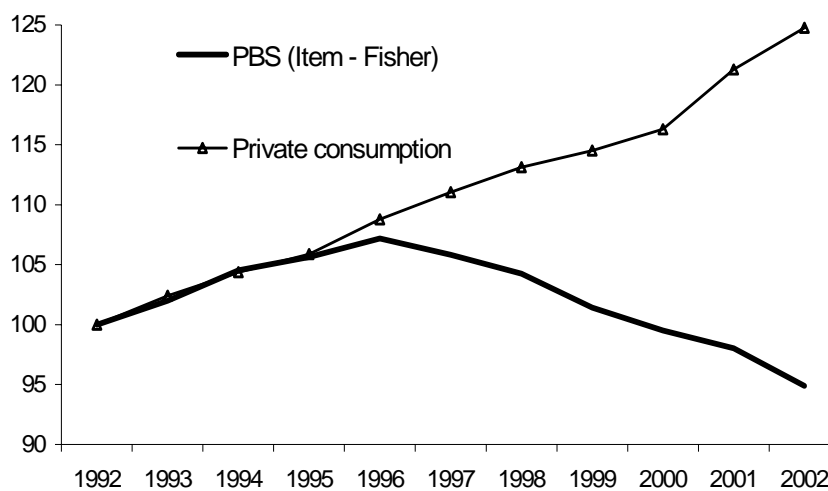
My colleague Kim Sweeny at the Centre for Strategic Economic Studies has examined some of these matters in detail, using the most detailed PBS data available and detailed IMS data for a number of countries (Sweeny 2003). This work follows the existing economic literature on index numbers and chained indexes. Two results of that work are reported on here.

The first concerns trends in the growth of the cost of the PBS over the past decade or so. It is often said, on the basis of trends in script numbers and average prices per script, that rising drug prices have primarily driven this growth, although the quantity of medicines used has also risen significantly. But this has no economic logic, for using scripts as a measure of quantity has no economic meaning. Medicines differ so much from one another that it makes no sense to add them up to create a quantity, and more

than it makes sense to add bicycles, cars, trucks and 747s up to get a number of transport vehicles. Other methods must be used.

The normal economic approach to is to create chain linked price indexes, and to use these price indexes to create measures of quantity. For example, such a method underlies the creation of price and quantity measures for Australia's national accounts. These price indexes are basically created by comparing weighted average prices levels for the medicines common to two adjacent years, and doing this for each pair of years to create a series of annual price changes. For the PBS, this approach is applied to about 3000 medicines at the item level.

Chart 2. PBS chained price index and consumption deflator (Index 1992 = 100)

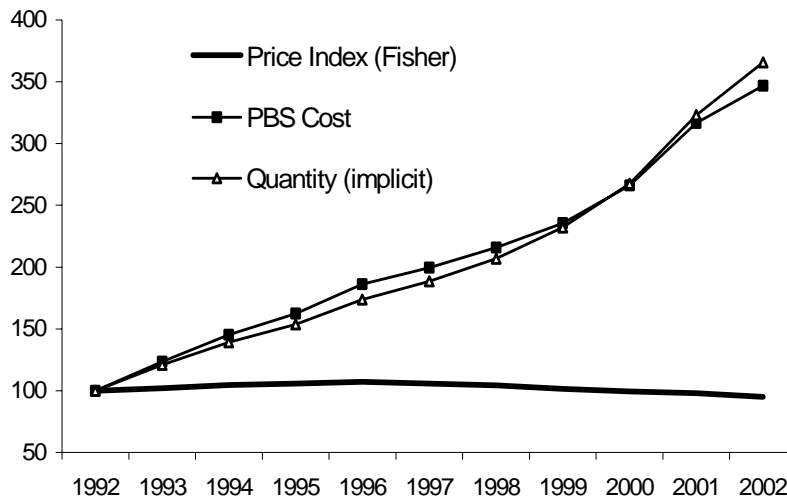


Source: Sweeny (2003).

The results of this work are summarised in Charts 2 and 3. Since 1996 aggregate PBS drug prices in Australia have fallen by about 10% in nominal terms (Chart 2), and by about 22% in real terms (relative to the personal consumption deflator). The overall drug price level is about 4% lower in 2002 than in 1992. As a result (Chart 3), all of the increase in the cost of the PBS is ascribable to the increased quantity of medicines used, and none to increasing prices. The timing of the fall in prices is broadly consistent with the progressive tightening of the PBS regime after the mid 1990s, and is confirmed by the concentration of the price falls in the highest cost areas, where policy action has been most concentrated.

These issues could, of course, occupy a paper or indeed a book on their own. My point simply is that key aspects of current PBS outcomes - low and falling prices for innovative drugs and uncertainty about listing timeframes and conditions - provide an inescapable background about discussions about the quality use of medicines. At the same time, the fact that we seem to have a low usage of generics, at relatively high prices, is suggestive of one way of increasing rewards to innovation while containing the cost of the PBS.

Chart 3. PBS cost-price and quantity trends (Index 1992 = 100)



Source: Sweeny (2003).

12. Major Limitations on Effective Application of Cost Effectiveness Analysis in Australia. The extensive use of cost effectiveness analysis in the PBAC is an important feature of the Australian system. But the application and value of such analysis in the PBS is currently compromised by many factors: the dominance of cost considerations and market power in subsequent negotiations; long delays in this process and lack of transparency; and the subsequent erosion of real prices agreed, through reference pricing based on therapeutic groups.

Australia pioneered the application of cost effectiveness analysis to the setting of the prices paid for medicines in a regulated system, and many resources and much expertise is devoted to such analyses today. While I would certainly not claim to understand fully the intricate processes of the PBS and its component institutions, it seems clear that the consistent application of cost effectiveness analysis is severely compromised by the factors mentioned above.

In terms of the determination of value within cost effectiveness analysis, two critical issues are the extent of the benefits included and the valuation methods employed for the benefits identified. Reflecting the segmented nature of Australian health institutions, only a limited selection of benefits are included for pricing purposes, and neither downstream health system savings nor economic benefits such as improved productivity are included. The value placed on a unit of benefit - such as a quality adjusted unit of life (QALY) saved - lies at the core of a cost effectiveness analysis. The PBS has declined to identify and use a specific value for a QALY, and the only study of which I am aware shows that a wide range of values, generally low by international standards, is implicit in its decisions (George et al 1998). It is hard to escape the impression that both of these policies are designed to preserve the role of the Government's market power in the final determination of prices.

3. The Use of Medicines

It was argued above that, around the world, the actual use of medicines is the forgotten dimension of the technological revolution in health. In some respects, Australia has addressed this issue better than most. It established a National Medicines Policy with a focus on QUM in 1992, and has created a number of institutions at or since that time to advance QUM in Australia. But no one could argue that the quality use of medicines has become central either to the public debate about health or to the practices of the health system in this country. In this section I seek to highlight the importance of QUM going forward, and to comment on some of the factors inhibiting its more effective application.

13. *QUM is Vital, and Could Be in the Interests of All Parties.* The quality use of medicines - ensuring that all Australians get the cost effective medicines, and only those medicines, that they need for optimum health outcomes - is of the utmost importance and can be in the interests of all parties. It will become even more central as the role of medicines increases further, and as they become more closely targeted to individual needs and to specifically defined disease states.

What, in practice, does the quality use of medicines mean? The definition of QUM in the National Strategy implies that there are at least five types of activity that would be inconsistent with quality use of medicines:

- utilisation of medicines for uses beyond (less valuable than) the indications for which PBS listing has been provided, where more cost-effective alternatives are available;
- other mistaken or inappropriate prescribing or use of medicines by health professionals, including those leading to adverse medicines events;
- non-compliance with prescriptions, and waste or other misuse of medicines by individuals.
- under-utilisation of medicines, through failure of an individual to be diagnosed with a condition for which medicines are available, or failure to be prescribed the appropriate drug; and
- delay in being able to access advanced medicines, for various reasons.

These activities involve failures of both medical effectiveness and failures of cost effectiveness. That is, people do not get or take the medicines that are best for them or use medicines that they do not need, waste medicines or use an expensive one for an indication where a cheaper one would be more cost effective. There seems to be little systematic evidence about these five activities in Australia or for that matter anywhere in the world. In particular, there is little quantitative data on health outcomes in the community, at the level at which medicines are actually prescribed and used. Nevertheless, there are signs that each of these five effects may be of substantial magnitude. Some of those signs are noted below.

Considerable concern has been expressed in Australia about the *use of medicines outside PBS indications*, in the sense that subsidised medicines are used for purposes other than those nominated by the PBS for price support. A number of drugs, of which the most notable example is Celebrex®, have seen sales after listing well in excess of the forecasts provided in listing documents. Corbett (2003) cites an AMA estimate that

proton pump inhibitors were intended for a target population of less than 35,000 people, but have actually been used by 177,000 people.

There is no doubt that 'leakage' of this type is a serious issue in a regulated system in which public funds are used to provide medicines to the public at well below market prices. But to say that actual usage was in excess of projected usage is not sufficient to establish leakage, and it seems that we have little hard information on the extent of the use of medicines outside indications in Australia.

In terms of *inappropriate prescribing or use of medicines*, one study of the National Health Service in the UK is reported as finding that more than 800,000 patients each year experience adverse drug reactions, leading to some 68,000 deaths and to 50,000 people becoming permanently disabled (IBMCS 2002). A US study found that in 1994 over two million patients were admitted to hospital because they were prescribed inappropriate drugs or experienced adverse effects from correctly prescribed drugs (Lazarou et al. 1998). Over 100,000 died as a result. It is widely believed that adverse drug events are increasing over time, as the number and complexity of medicines being taken increases.

There is some international literature on *non-compliance issues* (for an early review of some of that literature see McGavock 1996). A study by SRI Consulting in the US is reported to have found that one-third of prescriptions written never get filled and that non-compliance costs the US health industry almost US\$100 billion per year (*Market Letter* 1999). The report argues that 10% of all admissions to hospital and 25% of admissions to nursing home are directly related to non-compliant behaviour. More recent studies also suggest high levels of non-compliance. For example, a US study of patients with high cholesterol found that only 33% of those prescribed a statin were still taking one after twelve months, and after five years only 15% were doing so (Catalan and LeLorier 2000)..

In terms of *the under-utilisation of medicines*, take the example of cardiovascular disease, the greatest health problem in Australia as in most other Western countries. In 2000 it killed 49,700 people, making it responsible for about 40% of all deaths. Its two predominant forms are coronary heart disease (or ischaemic heart disease) and stroke and they accounted for 26,500 and 12,400 deaths respectively.

High blood pressure (or hypertension) is the most important contributor to the burden of both ischaemic heart disease and stroke, followed by physical inactivity and high blood cholesterol. Although a common problem and the largest single contributor to cardiovascular disease, most hypertension is untreated. The AusDiab study carried out in 1999-2000 found that some 30.6 % of males and 27.1% of females over the age of 24 had elevated blood pressure but only 11.5% of males and 15.3% of females were being treated for the condition. This is one of many cases in which better use of medicines would almost certainly achieve much improved health outcomes.

QUM can clearly mean different things to different people. But, from a welfare perspective, it is important that it be interpreted broadly, and not captured by any narrower conception. Thus QUM is about potential cost savings obtainable by ensuring that the subsidised use of medicines is confined to the indications for which they are funded, by it is not only about that. Equally, QUM may lead to increased sales of some

drugs that are currently under-utilised, but is certainly not only about that. Reducing the extent of drug related adverse events, ensuring better compliance with prescriptions and reducing access times are all important aspects of QUM. We all have our particular perspective on any issue, but national policy needs to reflect the broader view.

14. *Short-run Cost Emphasis and Low Prices for Innovative Medicines Distort QUM Focus*. Two important factors standing in the way of improved QUM in Australia are the dominance of short-run cost considerations in drug decisions at the Federal level, and the low and falling prices provided for innovative medicines. These factors mean that the objectives and incentives for decision makers at two key points – the Federal Government and the major companies – cannot be properly aligned with the quality use of medicines.

This final proposition emerges from the discussion above, and perhaps does not need further elaboration here.

4. The Foundations of Partnership

The foundations for any enduring partnership lie in realistic, shared understanding of the objectives of each of the partners. A starting point here is an acceptance that, in practice, most parties have multiple objectives and accountabilities. For example, as Dukes (2002) points out, drug companies are accountable both to their shareholders and to the community. Indeed, both accountabilities are becoming more intense, as capital market and competitive pressures increase and as the community becomes more informed and more demanding, both in terms of information and value. They also may be becoming more difficult for the companies to reconcile.

Similarly, governments must pursue the broad public interest, but they do so in practice with an acute sense of the immediate financial interests of their particular sphere of government and of their own political interests. Some of these conflicts between the long run public interest and immediate financial and political interests seem to have become more acute, at both federal and state levels, in recent years. Other parties represented at the NMPP meeting undoubtedly also have conflicting objectives as, for that matter, do university research centres.

In the present case, the foundations of partnership may lie in the overlap of agreed objectives at the general level. My view is that six objectives may be shared between the parties, and hence may provide a basis for partnership:

- commitment to improving health outcomes
- ready supply of best available medicines
- effective use of medicines to improve outcomes
- minimize overall health costs for a given level of health outcomes
- viable prices for innovative medicines
- building the medicines industry in Australia.

While many may agree on these as foundations for a real partnership, different items may well mean very different things to different people. But the essence of effective

partnership is finding ways to achieve the agreed broad objectives within the constraint sets of the individual parties.

It will be apparent from the foregoing material that I believe that two key issues that stand in the way of deeper cooperation around the quality use of medicines and a more effective system seem to be:

- the current funding structure, with virtually all of the costs of the PBS borne directly by the Federal budget, with individual major drug decisions heavily influenced by Treasury and Finance and determined by Cabinet; and
- the low and falling level of prices for within patent, innovative and cost effective medicines.

It is important to recognise the extent to which these stand in the way of a truly cooperative National Medicines Policy. Health policymakers will not be able to pursue QUM approaches adequately if they are being driven by short run financial rather than long run health outcomes and cost effectiveness objectives. Pharmaceutical companies will be under pressure to maximise revenues, and to make at most tactical commitments to QUM, if they and their head offices see pricing levels that are low by international standards and falling over time.

It is not the purpose of this paper to address ways in which these roadblocks can be removed, in a manner consistent with the broader objectives noted above. However, my own view is that serious consideration should be given to two broad approaches:

- creation of a separate, independent agency, to which the Medicare levy is assigned, which is responsible for funding and coordinating the cost effective quality use of medicines; and
- returning to cost effectiveness pricing, at realistic and sustained price levels, for innovative medicines, with an increased use of generics, at lower prices, for off patent molecules.

5. Some Forms of Cooperation

In this overall context, some brief comments are made on four areas in which stakeholders might cooperate in the immediate future.

Measuring health outcomes and monitoring the actual use of medicines

In the discussion above, we identified five types of activity inconsistent with the quality use of medicines: over-utilisation of medicines, inappropriate prescription or use within the health system, waste or other inappropriate use by individuals, under-utilisation of medicines and delays in access to advanced medicines. Because QUM is not, at the present time, a central focus of our health system there is little real information available on the extent or incidence of any of these five activities. Detailed data are collected about the performance of medicines in clinical trials, about financial transactions in the health system and about the incidence of disease and the causes of death. But each of these data sets are collected independently by a particular segment of

the health system, many are subject to severe confidentiality restrictions and none provide any real evidence on the quality use of medicines.

For example, massive data sets are assembled internationally, through clinical trials, about the safety and efficacy of drug candidates under control conditions. In Australia this information is marshalled by the companies and analysed in detail by the PBAC. But there is little evidence collected about the actual effectiveness of medicines under the conditions of real life and most of it is provided under strict confidentiality restriction.

Taking QUM seriously means taking seriously the data requirements for understanding the five types of activity, and for tracking trends in the different components of QUM. Existing data sources can surely be used to this end, but quite new collections would undoubtedly be necessary. A major, cooperative study to define the information requirements for an extensive application of QUM in Australia would be worth serious consideration.

Effective use incentives in pricing agreements

If we are to pursue the quality use of medicines systematically in Australia, it would be logical for appropriate incentives for QUM to be incorporated in the pricing agreements put in place. There has already been much discussion of one aspect of this, in terms of financial risk sharing through price-volume agreements and related arrangements. There also have been some examples of broader pricing agreements including QUM activities, including cases in which the incentive relates to the fact of listing on the PBS, rather than the price at which the drug is listed.

In a price-volume agreement, the PBS negotiates the prices it pays manufacturers for medicines partly on the basis of the anticipated utilisation of those medicines—generally the larger the anticipated market, the lower the price paid. In cases where there is question about the extent of the market for a drug, or where large volumes of sales are expected, the PBS has sought to establish agreements with manufacturers to vary the price it pays in the light of actual market variations. An example of a more broadly based approach is the targeted asthma drug monteleukast (Singular®), where a targeted QUM program, delivered by the National Asthma Council and funded by the drug company, was put in place as a condition of PBS listing.

While it is always sensible to use economic incentives to achieve social goals as far as possible, there are many issues to be addressed in a cooperative fashion here. Much of the discussion of risk management is really about financial risk management, that is about containing the cost of medicines to the PBS. While this will always be a valid concern, if the use of incentives is tied to this goal only the true QUM focus will be distorted. It is a complex task to think through how economic incentives could engage the resources of the supplier companies, in partnership with other groups, to advance the various aspects of QUM. But it is one that would be very worthwhile undertaking.

Defining the roles of all partners in QUM

Many different players are involved in the use of medicines, from the individual patient, the doctor and the pharmacist to the pharmaceutical company and the relevant government agencies. Each player has a different perspective on QUM and specific individual or corporate interests, while still playing an important role in the complex social system of health provision. This is classic coordination problem, and addressing it must be one of the central functions of a national QUM strategy.

Public policy initiatives to achieve quality use of medicine

The bottom line question is, in many ways, how public policy can intervene, while respecting the rights and freedoms of individual parties, to advance QUM. A good deal of thought has undoubtedly been given to this in Australia, in part through institutions such as PHARM and the National Prescribing Service. While aware of the complexity of these matters, I make no claim to be fully informed about progress made to date. There would seem to be value, however, in some more structured forum, where innovative thinking about how public policy can advance the various dimensions of QUM can be shared, and developed further.

6. Conclusion

There are two polar approaches to the situation that countries must address in the emerging world of the proliferation of research-based medicines. One is to treat medicines as commodities given from abroad, and to extract maximum social value for minimum cost from those commodities. Our friends from across the Tasman are taking this path, and the central mechanism involved is familiar enough: reference pricing, including generics, across a broadly based therapeutic basket, and aggressive competitive processes (such as tendering for the full market) for generics. The interaction of low prices for generics and the reference pricing system drives the overall price structure down, but leads to a progressive withdrawal of the international industry from both health and industry issues.

The other path is to engage with innovation, and to work systematically with all stakeholders to participate in the development of targeted new medicines and to ensure their early introduction and quality use within the country. This should involve, *inter alia*, a major national partnership program to ensure that all Australians have access to the medicines that they need, and that there is quality use of those medicines by them and by the health system as a whole. This path certainly offers better health and economic outcomes, and probably eventually lower health costs for a given level of health outcomes. But it does require some fundamental changes in the way we do things.

My hope is to see Australia embark with serious intent on the latter path. Australia, small country though it is, has pioneered many things over the past century, from the eight hour day and major social security programs to road safety measures and cost effectiveness analysis in drug pricing. The very fact of this meeting suggests that it has the chance to be a pioneer in the brave new world of the quality use of a wide range of targeted medicines.

References

- Australian Institute of Health and Welfare (AIHW) 2002, *Health Expenditure in Australia 2001*, Canberra.
- Australian Institute of Health and Welfare (AIHW) 2003, *Health Expenditure in Australia 2002*, Canberra.
- Catalan, V. S. and LeLorier, J. 2000, 'Predictors of Long-term Persistence on Statins in a Subsidised Clinical Population', *Value in Health* vol 3, pp 417-426.
- Commonwealth of Australia 2002, *Intergenerational Report 2002-03*, Budget Paper no. 5, Canberra.
- Corbett, J. 2003, 'Because it Matters, Big Time', paper presented at the conference 'The Future of the Pharmaceutical Benefits Scheme: Sustaining and Improving Australians' Access to Quality Medicines', PBS, Sydney, 11-12 September.
- Datamonitor 2002, 'The New Generation Blockbusters', May, 6 & 26.
- George, B., Harris, A. and Mitchell, A. 1998, 'Reimbursement Decisions and the Implied Value of Life: Cost effectiveness analysis and decisions to reimburse pharmaceuticals in Australia 1993-1996', in Harris, A. (ed), *Economics and Health: Proceedings of the 19th Australian Conference of Health Economics*, University of New South Wales, Sydney, pp 1-17.
- Houghton, J.W. 2003, *Australian ICT Industries Update 2003*, sponsored by the Australian Computing Society, Centre for Strategic Economic Studies, Victoria University, Melbourne.
- IBM Global Services (IBMGS) 2002, *Pharma 2010: The Threshold of Innovation*, available at http://www-1.ibm.com/services/strategy/e_strategy/pharma_2010.html
- Lazarou, J., Pomeranz, B. et al. 1998, 'Incidence of adverse drug reactions in hospitalised patients: a meta-analysis of prospective studies', *JAMA*, vol. 279, no. 15, pp. 1200-1205.
- Lichtenberg, F. 1996, 'Do (more and better) drugs keep people out of hospitals?', *American Economic Review*, vol.86, no. 2, pp. 384-389.
- Lichtenberg, F. 2003, 'The Impact of New Drug Launches on Longevity: Evidence from Longitudinal, Disease Level Data from 52 Countries, 1981-2001', NBER Working Paper no. 9754, NBER, Cambridge, Mass.
- Market Letter: The Authoritative Newsletter for the World Health Care Industry* 1999, 'Non-compliance costs almost \$100 billion pa', IMS World Publications, London, 21 June.
- McGavock, H. 1996, *A Review of the Literature on Drug Adherence*, Royal Pharmaceutical Society of Great Britain, London.
- Nordhaus, W. 1999, 'The Health of Nations: The Contribution of Improved Health Standards', paper presented at the conference "Economic Returns of Medical Research", Lasker/Funding first, at <http://www.laskerfoundation.org/reports/reports.html>
- Sweeny, K. 2003, 'Price and Quantity Trends in the Pharmaceutical Benefits Scheme', Pharmaceutical Industry Project Draft Working Paper, Centre for Strategic Economic Studies, Melbourne.
- Wanless, D. 2002, *Securing our Future Health: Taking a Long-Term View*, HM Treasury, London, available at http://www.hm-treasury.gov.uk/consultations_and_legislation/wanless/consult_wanless_interimrep.cfm