Funding arrangements for pharmaceuticals: can economic evaluation promote efficiency?

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The context

The media coverage of health resource allocation, even in the "quality" press, is generally simplified, reducing the issue to winners and losers in political terms, and rarely providing any in-depth analysis of policy initiatives. Policies are generally analysed in terms of how difficult they will be for politicians to "sell" and the effect they will have on electoral success rather than the impact they will have on the health of the population, on resource allocation or on equity of access (Haas et al. 2001). The recent media attention on the Pharmaceutical Benefits Advisory Committee is a case in point.

Since 1993, Australia has had an internationally unique process for listing new pharmaceuticals for subsidised access, based on cost-effectiveness. While there has been considerable interest in it within the health policy and health economics fields, both here and overseas, until recently it has received little discussion in the media. In the last year it has been the subject of considerable attention, first, because of legal dispute between the government and pharmaceutical companies over failure to list particular drugs, and then because of conflict between the government and members of its own advisory body, the Pharmaceutical Benefits Advisory Committee (PBAC). The nature of the media reporting, focussing on conflict between individuals, on courtroom battles and on individual "losers" from listing decisions makes it difficult for anyone outside of the policy process to understand the principles on which it is based or to make an informed assessment of the system.

Pharmaceuticals are a major component of health costs. In 1999-00, Commonwealth government expenditure on pharmaceuticals subsidised under the Pharmaceutical Benefits Scheme (PBS) was $3.49 billion (Department of Health and Aged Care, 2001). Expenditure on pharmaceuticals represents approximately 13% of the Commonwealth health budget (AIHW, 2000). The average annual growth in expenditure on benefit-paid pharmaceuticals in the decade to 1997-98 was 7.5% (AIHW, 2000). As with many other technologies in health care, the potential for growth in expenditure is very large. New and increasingly expensive drugs are being developed to treat more and more conditions. Without subsidy from government, access to drugs would be extremely inequitable. Good management of pharmaceutical costs is therefore important for any country.

Australia has been a world-leader in using relative cost-effectiveness compared with existing therapies as a criterion in determining whether a new pharmaceutical will be subsidised by government (by inclusion on the PBS). This paper aims to provide a brief description of Australia's regulation of pharmaceuticals, and to provide some commentary on the strengths and weaknesses of the system. In the current media debate there has been relatively little discussion of the strong regulatory framework for pharmaceuticals in Australia, or the economics principles underlying it.

Overview of the PBS: How do new drugs get listed for subsidised access?

The PBS is an open-ended benefit scheme providing subsidised access to pharmaceuticals for patients in the community and in private hospitals. For a drug to be listed on the PBS it must first be approved by the Therapeutic Goods Administration (TGA). The role of the TGA is to determine whether a drug is safe and efficacious. Once a drug is registered with the TGA it can be marketed in Australia, but patients will have to pay the full price, unless it is recommended for listing on the PBS by the PBAC. Under the legislation, the Minister for Health approves drugs for listing based on recommendations made by the PBAC.

The PBAC considers the "effectiveness, cost-effectiveness and clinical place of a product compared with other products already listed in the PBS for the same, or similar indications" (Commonwealth of Australia, 1995, p2), or, where there is no comparable drug already listed, compared with standard medical care. Submissions to the Pharmaceutical Benefits Advisory Committee (PBAC) must include an economic evaluation to seek listing of a new drug, a significant change to the listing of a currently restricted drug, or to seek a price premium for a currently listed drug. Submissions are generally made by the manufacturer, but may be made by other interested parties, including health professionals or private individuals. There are published guidelines for preparation of submissions, and, in particular, how to conduct and present the economic evaluation for the submission. The PBAC has an Economics Sub-Committee (ESC) whose role is to assess the economic evaluations included in submissions, as well as to provide advice on economic evaluation methods. The guidelines ensure that, as far as possible, drugs are evaluated on the same basis, and that the methods used in estimating relative cost-effectiveness of different therapies are transparent.

When a submission is made to the PBAC, it must include a requested price. This price is an input to the economic evaluation undertaken as part of the submission. However, it is not necessarily the price at which the drug, if approved, will finally be listed. Decisions about pricing are made by the Pharmaceutical Benefits Pricing Authority (PBPA), based on advice from the PBAC. The role of the PBPA is to negotiate a price with the manufacturer that ensures a secure supply of the drug for the Australian population at a reasonable price. The process of price setting takes into account the cost-effectiveness of the drug, based on advice from PBAC, but also other factors such as the cost of manufacture, the price of the drug in comparable countries and productive activity by the manufacturer in Australia (for example, new investment in Australia).

Does the system lead to better use of resources?

Two questions can be asked about the emphasis on economic evaluation in the process of listing new drugs. First, is economic efficiency an appropriate criterion for determining what drugs will be available to whom? Second, does the process contribute to improved efficiency?

While the PBS may be an open-ended scheme, overall government resources to spend on health are constrained. Additional spending in one area means health gain will be forgone elsewhere. If the objective of the health system is to maximise health gain, efficiency is an appropriate criterion to guide spending, and economic evaluation provides the necessary information. When resources are limited, health gain (or benefits) will be maximised by allocating them to the alternative with the lowest opportunity cost. This is the principle underlying economic evaluation: to find the intervention from the available alternatives with the lowest cost per additional unit of health outcome (measured in, for example, lifeyears saved). Because health care interventions are generally complex, affecting quality of life and survival, outcomes are often measured in terms of a measure such as quality adjusted life years (QALYs). The PBAC guidelines allow for the use of QALYs or similar measures.

The proposition that "value for money" or relative cost-effectiveness is appropriate for governments considering how to spend health resources seems relatively uncontroversial, but it has been the basis for much of the criticism levelled at the government over the PBS. When a new drug is not approved for listing on the basis that it is too costly given the health benefit it provides, there is clear tension between the potential benefit to some individuals, and the opportunity cost to society overall. It is common for governments seeking to use efficiency as an explicit basis for allocating health care resources to encounter opposition in the form of individually worthy cases (leading to the "rule of rescue"), or in terms of a trade-off between equity and

efficiency (Hall and Haas, 1992). In the case of pharmaceuticals, cost-effectiveness is not the only factor to be taken into account by the PBAC, but it is a major component of the decision. Without the principle of efficiency to guide decision making, scarce resources will still need to be allocated between alternatives, and the basis for decision making is likely to become ad hoc.

Putting the principles of economics into practice in decision making, even in a formalised process as in the PBS, is difficult. Using economic evaluation to assist decision-making will promote efficiency, if, as a result of undertaking the evaluation resources can be directed away from the less cost-effective alternative to the more cost-effective alternative. However, because the PBS is an open-ended scheme, there is no direct relationship between the recommendations of the committee and how resources are expended. Even if all new drugs listed were more cost-effective than existing alternatives, the process of changing prescribing patterns is slow, and drugs will not always be prescribed solely for the indications listed or the population group with a demonstrated benefit. In reality of course, the drugs already listed on the PBS will vary enormously in terms of cost-effectiveness, and similarly, the cost-effectiveness of new drugs will be quite variable (George et al. 1999).

The lack of an explicit budget constraint for pharmaceutical expenditure poses a more fundamental problem. Even given the variability in cost per lifeyear saved of new products recommended by the PBAC, it is likely that over time, a signal will be sent to the manufacturers about the threshold price at which government is willing to "purchase" more lifeyears. That is, it is likely that an implied threshold cost-per-year saved can be inferred from successive decisions. If prices of pharmaceuticals were determined through a competitive process, this may not be a problem. In fact the price is first proposed by the manufacturer, built into the economic evaluation, and then used as the basis for negotiation between manufacturer and government. The marginal cost of supplying pharmaceuticals to Australia, will, in general be relatively low. Manufacturers are unlikely to be seeking to recoup research and development costs in the Australian market. Further, manufacturers will, to some extent have monopoly power (matched by the monopsony power of government). Thus, it is potentially feasible for the manufacturer to choose a price that provides a favourable cost-effectiveness result, but increases profits for the company. An explicit threshold cost per lifeyear saved is "a recipe for uncontrolled growth in expenditure" (Gafni and Birch, 1993, p913), but even an implicit threshold (which is almost an inevitable result of the process) has its risks.

Perhaps the biggest challenge for governments seeking to use economic evaluation to assist decision making in this way is to ensure that the process does not lead to improvements in technical efficiency across a very narrow spectrum of interventions.

Economists make a distinction between technical efficiency and allocative efficiency. Technical efficiency is concerned with finding the lowest cost way of producing a given output, whereas allocative efficiency is concerned with deciding what outputs to produce. The use of economic evaluation in assessing new products for listing on the PBS is answering questions of technical efficiency. Cost-effectiveness analysis cannot answer the allocative efficiency question of whether the additional health gain is worth the additional benefits forgone elsewhere, that is, whether the marginal benefit to society outweighs the marginal cost. The PBAC has recently examined the use of cost-benefit analysis in evaluating pharmaceuticals. However, the issue of placing monetary values on benefits remains controversial.

Even the application of the technical efficiency criterion is necessarily limited. The comparator used in undertaking economic evaluations for PBAC submissions is generally the drug the new product is most likely to replace in treatment. This answers the question of whether the new product is more efficient than the currently listed product at providing health gain for people with a specific condition. It cannot answer the question of whether allocating the required resources to pharmaceutical expenditure rather than some other area of the health budget is likely to provide the most benefit. Non-pharmacological interventions will generally only be the chosen comparator if there is no existing treatment on the PBS. There are unlikely to be any sponsors for economic evaluations of, for example, health promotion interventions.

The establishment of the Medical Services Advisory Committee is a welcome step extending the requirement to demonstrate relative cost-effectiveness to new services funded under the Medical Benefits Schedule, but in practice this is a similarly limited application of efficiency to health care resource allocation. The more fundamental inefficiencies in the health system are difficult to address when there are multiple sources of funds

for health services, with some services being funded on an open-ended basis, and others being funded from a capped budget.

Nonetheless, the PBS provides one example of the explicit and consistent application of economics principles to funding of health services. The guidelines for economic evaluation, themselves, serve as a model that can be applied across different health services. It is important to recognise that there are risks to explicit use of cost-effectiveness principles in one area of health care resource allocation, especially in a complex and fragmented health system with public and private provision.

Pharmaceuticals present particular challenges because of the interaction between industry and government. Other countries have adopted the use of economic evaluation of public funding of pharmaceuticals, although few systems are as systematic or formalised as in Australia (Salkeld et al, 1998). The Australian approach deserves support from the health system, but this support should include critical analysis of the incentives that are built into the processes.

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