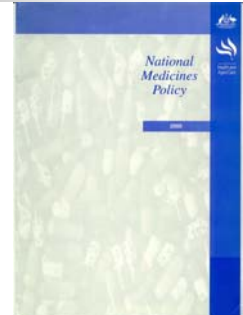


Pharmaceutical Benefits Scheme Rationale and Process

Karen Binnekamp
Secretary, Pharmaceutical Benefits
Advisory Committee

Australia's National Medicines Policy: 2000

- To meet medication and related service needs, so that both optimal health outcomes and economic objectives are achieved



National Medicines Policy Objectives



2nd Intergenerational report

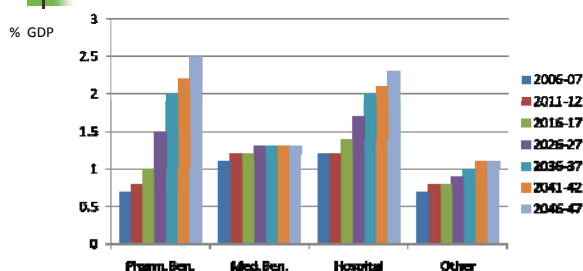
- "The main spending pressures for the Government will be in health, aged care and the Age Pension. These areas will be put under increasing pressure by demographic change. Other factors, such as new and improving technology, are also projected to increase costs, particularly in health."

Treasurer 2 April 2007

Intergenerational Changes

2006-07 to 2046-47

Projected Pharmaceutical Benefit Expenditure In Relation to Other Health Services [As a Proportion of Gross Domestic Product]



Source: Intergenerational Report 2, Australian Government – The Treasury April 2007.

Cancer Chemotherapy

- 34% increase in new cancer cases between 1991 and 2001, as a direct consequence of population ageing
- Cancer survival rates have increased by 1% p.a.
- More demand for second and third line therapies

SUBSIDY OPTIONS

- Fund all registered drugs at the price requested
- Fund drugs at price requested but total drug budget capped
- Fund drugs at price requested but total cost of drug/drug class capped
- Fund registered drugs at a percent of requested price e.g. 50%

SUBSIDY OPTIONS

- Fund only one drug of a class at a price accepted by tender
- Evaluate cost versus benefit in an uncapped system

WHICH OPTION PROVIDES BEST EQUITY AND RECOGNITION OF OPPORTUNITY COST IN HEALTH CARE?

Australian approach to subsidy

- Evaluate cost-effectiveness in an uncapped system

Or another way to say this:

- Pay for the outcome rather than the medicine

The PBAC process

1. Define the clinical place of the new drug,
2. Determine what the new drug will replace,
3. Assess how the new drug compares in terms of effectiveness and safety,
4. Assess the incremental cost of any efficacy or safety benefit the new drug confers,
5. Examine the likely overall cost of subsidy.

Cost effectiveness

- incremental cost effectiveness ratios are expressed as the cost per health outcome
- the type of outcome will be different for medicine, patient and disease groups

Economic evaluation

Cost Minimisation

- for drugs that have the same outcome

Cost effectiveness

- for drugs that have a clinical advantage
- measured in natural unit (eg life-years gained or points of BP reduction)

Economic evaluation

Cost utility analysis

- health outcomes rated by preference strength (e.g. healthy years or quality adjusted life years QALY).
- Output is cost per unit of preference state.

Modelled economic evaluation

- estimation of remote outcomes, final outcome, cost offsets

Cost-Effectiveness requirements

- Is the drug to be targeted for optimal cost effectiveness and is this pre-defined?
- Health gain measured in QALY - requires assessment of utility evaluation
- Costs and cost offsets - what are they and how are they measured
- What are the uncertainty factors - are they clinical and/or economic?

COST EFFECTIVENESS

CAN BE IMPROVED BY

- Improving the outcome through targeting of patients and/or use of continuation rules
- Increasing cost offsets
- Reducing price

Timing

- PBAC meets three times per year
- Submissions due four months prior to PBAC
- Listing commences approx. 4½ months after PBAC recommendation
- Only exceptions are drugs costing over \$10 million per year, where Cabinet consideration required

Registration vs subsidy

- Registration generally considers absolute quality, efficacy and safety
- Subsidy considers comparative clinical benefit and cost of any additional benefit

Future challenges

- Demand for health services will continue to increase
- The cost of health care will continue to increase
- The pressure on budgets (governments, institutions and individuals) will continue to increase
- How to promote disease-prevention with the same profile as disease-management

Future challenges

- Greater pressure on the price of pharmaceuticals-downwards and upwards
- Need to maintain evaluation of health outcomes as the purchasing criterion
- Greater use of pharmacogenomics to target therapy to achieve cost effectiveness
- Increasing need for the evaluation of “value for money” in practice

Drug assessment challenges

- Greater use of surrogate outcomes with resultant uncertainty about “patient-relevant” outcomes and cost effectiveness
- A growing demand for early access to new drugs
- Management of uncertainty: risk-sharing, post-funding data collection, coverage with evidence development

Surrogate Outcomes

- A clinical endpoint is a characteristic or variable that reflects how a patient feels, functions or survives
- A surrogate endpoint is a biomarker that is intended to substitute for a clinical endpoint.
- A surrogate endpoint is expected to predict clinical benefit on the basis of epidemiologic, therapeutic, pathophysiologic or other scientific evidence

Surrogate outcomes for survival

- Time to disease progression
- Progression free survival
- Stable disease
- What is the relationship between these surrogates and survival for a particular disease
- How certain is cost-effectiveness analysis when based surrogate end-point?

Surrogate Issues and Challenges

- use of surrogate endpoints in oncology particularly causing PBAC issues
- the link between the surrogate outcome and patient-relevant outcome(s) is often highly uncertain
- this leads to uncertain cost-effectiveness and if the price is high...

Opportunity Cost

Which is the better use of public funds?

- \$200 million a year for a drug which increases median survival by 3 weeks
- or
- \$200 million to improve screening for early detection or to improve palliative care

EQUITY AND SUSTAINABILITY

- "Equity" requires serious public financial commitment
- "Sustainability" requires effective expenditure management

HAVE WE GOT IT RIGHT?

"We cannot change the direction of the wind, but we can adjust our sails to ensure that we go in the direction that we want"

Specific issues in Oncology

- Small incremental benefits at large cost
- Use of surrogate outcomes
- Short term trials with high uncertainty
- Early interim analysis and open label design
- Cross over designs

Specific issues in Oncology

- Continuation beyond progression in practice, although trials ceased therapy on progression
- Incremental creep in cost via incremental benefit

Specific issues in Oncology

- Variation in opinion re comparator arms –this also an issue in cost offsets eg number of transplants avoided
- Use of high cost drugs in combination with a small incremental benefit
- Utilities measure towards end of life-is quality of life relevant in late disease
- What constitutes best supportive care?
- High uncertainty in modelled projection from surrogate to survival and in ICER

THE Hon RALPH HUNT MINISTER OF HEALTH

“WHATEVER DECISIONS ARE TAKEN
WILL REFLECT THE GOVERNMENT’S
DETERMINATION TO GET MORE VALUE
FOR THE DOLLARS SPENT ON HEALTH
CARE”

16 April 1978

THE WALL STREET JOURNAL March 7,2008

- “If the FDA has been given the power to make decisions that have such huge ramifications,it must be accountable for the cost-benefit ratio of these decisions.In this case,a study showed there was no survival benefit yet the costs will be billions of dollars per year.Is there any wonder why our health care expenditures are expected to double to over \$4trillion within 10 years”

HEALTH TECHNOLOGY ASSESSMENT (HTA)

- HTA involves the medical, social, ethical and economic implications of the development, diffusion and use of a health technology. HTA has been positioned as a ‘bridge between scientific evidence and the needs of policymakers’

HEALTH TECHNOLOGY ASSESSMENT

- The major aim of health technology assessment for registration is to provide consumers with safe and effective drugs
- The major aim of assessment for subsidy is to ensure that the cost effectiveness of a drug represents “value for money” taking into account opportunity costs

CONTEXT OF THE ASSESSMENT FOR THE CONSUMER

- A therapeutic option decision might be very much influenced by the comparison of studies using placebo as comparator relative to those using an active comparator which may be a treatment option. The comparative risk/benefit/cost options may be different in the two scenarios.

The Commonwealth Fund _July 2007-US Congress Health Care Bills

- creation of a Center on Comparative Effectiveness and Evidence-Based Decision-Making to promulgate information on comparative effectiveness of prescription drugs, devices, and procedures as well as adequate funding of health services research through the Agency for Healthcare Research and Quality;

Cost-Effectiveness requirements

- Comparative –what is comparator?
- Outcome measure-surrogate for patient relevant outcome? Is there a biological plausability?
- Is study design appropriate for subsidy considerations-impact of truncated studies or open label phases

Restrictions

- restrictions are used to target drugs to those indications, patient groups or clinical settings which achieve the optimum clinical and cost effectiveness
- a drug may be acceptably cost effective when used for one indication or patient group but not cost effective when used under other circumstances

Registration versus Subsidy

- "...so there is an increasing obligation for the developers of new treatments to provide evidence on a broader range of questions and outcomes in addition to the efficacy and safety data required by licensing authorities"

Freemantle et al Pharmacoeconomics 2005;23(8):747-754

COST OF NEW AGENTS

- A SENIOR INDUSTRY EXECUTIVE*
"WILL NEW PRODUCTS COMING DOWN THE R&D PIPELINE BE WORTH MARKETING EVEN IF THEY ARE APPROVED BY THE REGULATORY AGENCIES? IF THE PRICE IS NOT RIGHT WHAT CHANCE DO THEY STAND OF BEING USED"

* Scrip Magazine ,Feb 2004

INNOVATION-WHAT IS IT?

Canadian definition

- An innovative product is defined as the first drug product to treat effectively a particular illness or which provides a substantial improvement over existing drug products

INNOVATION- NIHCM Foundation Report 2002

- "Despite the enormous contribution these medicines have made to enhancing health, the quality of pharmaceutical innovation varies widely. It ranges from breakthrough treatments for life threatening diseases to minor modifications of drugs that have been on the market for some time"