

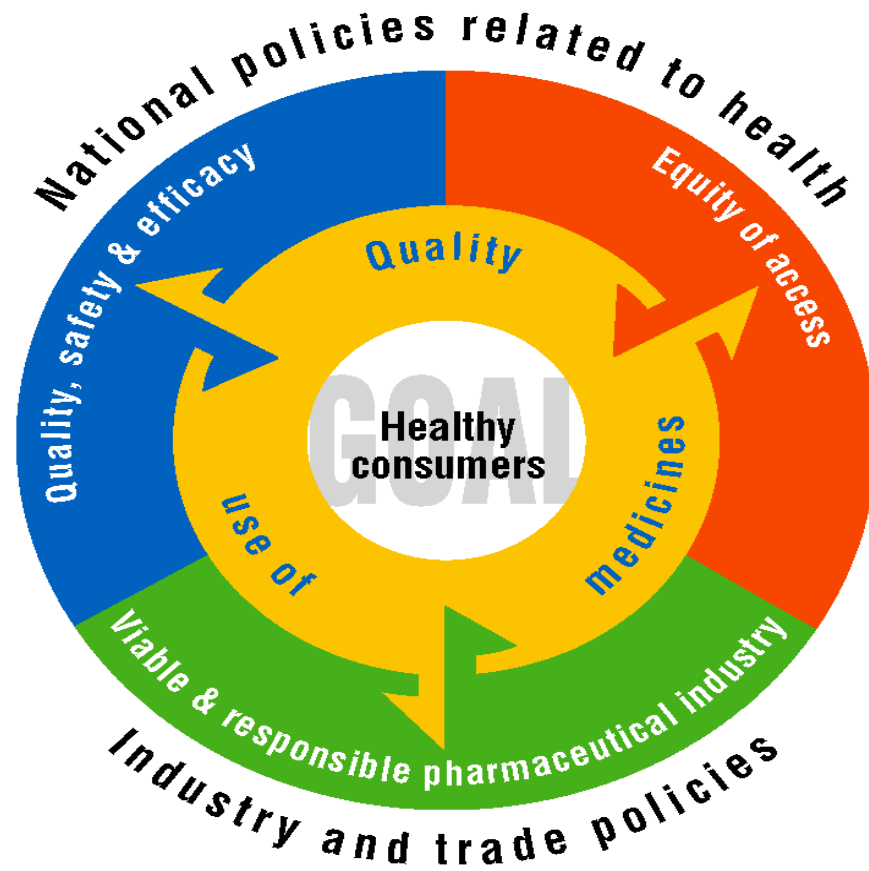


# THE PBS AND ITS PROCESSES

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Lloyd Sansom  
Chair, PBAC

# QUM and the National Medicines Policy



# Pharmaceutical benefits scheme



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- **providing timely, reliable and affordable access for the Australian community to necessary and cost effective prescription medicines**



# National medicines policy

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- **In attempting to balance health needs and responsible fiscal discipline, the partners need to address the following issues**



# Access to medicines

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- **financing and supply arrangements for medicines optimise health outcomes and represent value for money**
- **all partners take adequate responsibility for achieving value for money**



# Access to Medicines

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- **financing arrangements for medicines avoid incentives for cost-shifting between levels of government or other funders, or other perverse incentives**

# Assessment of evidence



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- **sponsor asked to categorise against the main comparator**
- **having significant clinical advantages**
- **being no worse in effectiveness and toxicity**
- **being less effective and less toxic**

# Cost effectiveness



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- **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**
- **PBAC actually recommends the purchase of outcomes**

# Economic evaluation



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**Cost Minimisation**- used when drugs have the same outcome. Ensure that the new drug is no worse than comparator (ie therapeutic equivalence).

# Economic evaluation



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- **Cost effectiveness.... clinical advantage measured in natural unit (eg life-years gained or points of BP reduction or other surrogate measure)  
ie cost per unit of effect**

# Economic evaluation



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- **Cost utility analysis**.... health outcomes rated by preference strength (eg 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

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- CAN BE IMPROVED BY
- *Improving outcome-targetting of patients and/or use of continuing rules*
- *Increasing cost offsets*
- *Reducing price*

# Restrictions



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- **restrictions are used to target drugs to those indications, patient groups or clinical settings which achieve the optimum clinical and cost effectiveness. This may reflect trial entrance requirements where attributes may be a treatment-effect modifier**

# Restrictions



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- a drug may be acceptably cost effective when used for one indication or patient group but not cost effective when used under other circumstances
  
- **IS PHARMACOGENOMICS THE ANSWER?**

# Relevant factors



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## Readily Quantifiable

- comparative health gain
- affordability
- financial implications for PBS
- financial implications for Govt health budget
- comparative cost effectiveness

# Relevant factors



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## Less Readily Quantifiable

- severity of condition treated
- presence of effective alternatives
- ability to target therapy to those likely to benefit most
- uncertainty
- equity
- development of resistance
- Govt health priorities and other relevant factors



# PBAC/ATAGI INTERACTION

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- ATAGI will continue to be responsible for the development of the National Immunisation Handbook
- PBAC will seek technical information from ATAGI as appropriate. This advice will be made available to the sponsor prior to the PBAC meeting as for other information provided to the PBAC

# New agents with limited data



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- more new drugs receiving marketing approval with limited data
- increasing use of modelling to predict benefit (eg prolongation of life based on unsubstantiated assumptions)
- increasing uncertainty in magnitude of benefit and thus in CE
- need to develop risk sharing arrangements and regular review of new listings (eg at 1,2 and 5 years)

# NEW AGENTS WITH LIMITED DATA



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- **Uncertainty in the pharmacoeconomic models used to predict benefit beyond the trial data eg prolongation of life based on unsubstantiated claims. Increasing uncertainty in magnitude of benefit and thus in CE eg 90% CI for cost/QALY ranging from \$30,000 to \$1,300,000**



# "THE PRICE TAG ON PROGRESS"

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- 'AS A SOCIETY, WE ARE RELUCTANT TO SYSTEMATICALLY DENY ACCESS TO EXPENSIVE TREATMENTS THAT EXTEND LIFE BY A FEW WEEKS, BUT THE MORALITY OF REFUSING TO MAKE DELIBERATED CHOICES IS ITSELF QUESTIONABLE'

D Schrag, N Engl J Med July 2004



# Future

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- **The tension between health demand and the capacity to pay will continue to be a major issues for all societies in both developed and developing countries**



# Future

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- recognition of the potential conflicts between social, economic, health and industry policies and the need for development of a **“whole of government”** approach-  
**IS THE HEALTH BUDGET THE PLACE TO RECOUP R&D COSTS?**



# Future

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- The use of cost effectiveness as the basis for the purchase of new health technologies, including medicines, will expand internationally .
- Clinical trial design will need to change to reflect the need for cost effectiveness analysis



# Future

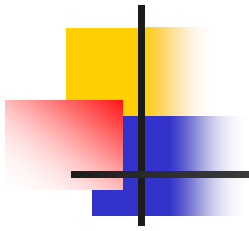
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- Greater emphasis on preventative health
- A very significant reduction in health burden by smoking cessation, exercise and diet. 30% of Australians are obese. Drug therapy alone is not the answer
- Increased emphasis on patients taking some greater responsibility for their own health



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“Equity requires investment,  
Sustainability requires  
management”  
(John Montgomery 2002)





# GUIDELINES FOR VACCINES

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# GUIDELINES FOR VACCINES

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- 1.1 *Pharmacological class and action-eg* description of vaccine, defining characteristics provide number, identification and amounts of antigens in the proposed vaccine
- 1.2 *Indications* eg NIP or PBS More specific considerations favouring a submission for NIP funding include



# GUIDELINES FOR VACCINES – NIP listing

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- (a) when the target is in a broader population where there is no need to assess risk factors or the assessment of risk factors at an individual level is straightforward
- (b) there is a reason to maximise population coverage due to severity or prevalence in an unimmunised population
- (c) The efficacy of the proposed vaccine is sufficient
- (d) there are putative advantages of increasing herd immunity

# GUIDELINES FOR VACCINES-PBS



## listing

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- PBS listing might be favoured when the proposed vaccine is "discretionary" for the majority of the population or the assessment of risk factors are less straightforward eg a where an assessment of immune system status is required



# GUIDELINES FOR VACCINES

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- This part of the submission must explain and justify proposed restrictions eg to certain populations, geographical distributions and ethnic groups and if a catch up program is requested
- The relationship between the proposed vaccine and currently available vaccines available on the NIP and/or PBS



# GUIDELINES FOR VACCINES

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- 1.3 *Treatment details* -proposed schedule including any new requirements likely to have financial implications
- 1.4 *Co-administered and substituted therapies* -is the proposed vaccine to be available as a substitute for existing product or to be added to the current arrangements for NIP/PBS?



# GUIDELINES FOR VACCINES

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- 1.5 *Main Comparator* -vaccine likely to be replaced-if there is no currently no vaccine available,then the main comparator will usually be standard medical management
- 1.6 *Differences between the proposed vaccine and the main comparator-main* differences in indications, contraindications, cautions, adverse drug effects



# GUIDELINES FOR VACCINES

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- *2. Data from comparative randomised trials for the main indication*
- *2.6 Analysis of comparative randomised trials-*  
If immunogenicity outcomes are the primary outcomes then the validity of these surrogate measures in relation to patient-relevant outcomes ,need to be established (Appendix O still being developed)



# GUIDELINES FOR VACCINES

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## DEMONSTRATING PROGNOSTIC VALIDITY ON IMMUNOGENICITY REQUIRE ANALYSIS

- (a) showing that a threshold level of antibody response predicts an extent of protection and thus a reduction in number of cases
- (b) assessing whether there is any limit to the duration of this effect or waning of effect over time
- (c) justifying claims that effectiveness is similar in primary and catch-up populations



# GUIDELINES FOR VACCINES

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- **3 Modelled Economic Evaluation**
- *3.2 Population used in the modelled evaluation* –the base case should be for the primary population
- *3.3 Approach used in the modelled evaluation* -there are generally two types of models used to estimate the epidemiological impact of vaccination programs-STATIC and DYNAMIC Models



# GUIDELINES FOR VACCINES

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- STATIC models are those in which there is a constant force of infection (probability per unit time that a susceptible person acquires infection) is favoured when the proposed vaccines is discretionary for the majority of the population .
- The DYNAMIC model is where the force of infection is a function of the number of infectious individuals in the population at that time



# GUIDELINES FOR VACCINES

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- 4 *Estimated extent of use and financial implications*