# INTRODUCTION TO PHARMACOECONOMICS

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MUSA
Medisyneverbruik in Suid-Afrika
Medicine usage in South Africa

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# Workshop objective & outline

- Objective:
  - To impart a basic understanding of the concept of pharmacoeconomics

- Outline:
  - The rationale and importance of pharmacoeconomic analysis
  - What is pharmacoeconomics?
  - Types of pharmacoeconomic analysis
  - Steps in conducting a pharmacoeconomic analysis
  - Application of pharmacoeconomic analysis

# Why pharmacoeconomics?

- Never enough resources to treat all potential patients
- Health care funders (governments, social security funds, insurance companies, medical aid) are struggling to meet rising costs
- Provides balance between needs and resources - allows decisions to be taken on who, how and when to treat
- A "dispenser of pharmaceutical justice" (Vella, 2010)
- Provides the means to quantify the value of the product to the rest of the health care system and to society as a whole.

# What is pharmacoeconomics?

- **Health economics** is about making choices between options, when there is scarcity of resources. It is fundamentally comparative, weighing the costs and benefits/outcomes of option 1 with those of option 2.
- **Pharmacoeconomics** is a branch of health economics that particularly considers drug therapy (pharmaceutical product/ therapy /services).
- Evaluate, measure and compare **input costs** (resources consumed) with its **outcomes** (consequences)

# Pharmacoeconomic costs and outcomes

- Input costs = expenses incurred in the provision of healthcare products and services. Four types:
  - **direct medical** costs like office visits, hospitalisations, any treatment costs; **direct non-medical** costs like transportation to get treatment;
  - **indirect** costs like missed work due to illness;
  - **intangible** costs like pain and suffering.

- Outcomes = Outcomes (ECHO) of pharmaceutical products/services. Three types:
  - **E**conomic: Total cost of treatment options, alternatives and the global effect on society
  - **C**linical: Medical events that occur as result of disease or treatment
  - **H**umanistic: Functional status, quality-of-life, including physical, emotional and social well-being

# Types of pharmacoeconomic analysis

- Four basic types:
  - Cost-minimisation analysis (CMA)
  - Cost-effective analysis (CEA)
  - Cost-utility analysis (CUA) and
  - Cost-benefit analysis (CBA)

- Quasi-health economic evaluations:
  - Cost consequence analysis
  - Cost-of-illness (COI) (Bootman *et al.,* 1996)

# Cost-minimisation analysis (CMA)

| When to use | Compares two or more alternative treatments that produce clinically equivalent outcomes. Analysis thus limited to costs. |
|---|---|
| Application | • Different dosage forms of a specific active substance van with equivalent outcomes<br>• Generics / Generic similar products<br>• Different treatment periods with equivalent outcomes (3 days IV vs 5 days oral)<br>• Treating patient w/same therapy in hospital vs home |
| Cost unit:<br>Outcomes unit:<br><br>Calculation | Monetary value<br>Natural units (equivalent)<br><br>(Cost of option A) - (Cost of option B) |
| Interpretation | Choose option with lowest cost |

# Cost-effectiveness analysis (CEA)

| When to use | Identifying a preferred choice among various possible alternatives |
|---|---|
| Application | Decide if outcomes are worth their corresponding costs relative to competing alternatives<br>• Therapies are less expensive and at least as effective as alternatives<br>• Therapies more expensive than alternative therapies with add. benefit worth the add. cost<br>• Therapies less expensive and less effective in instances where the extra benefits provided by the competing therapy not worth add. expense |
| Cost unit:<br>Outcomes unit:<br><br>Calculation | Monetary value<br>Natural units, e.g. weight gained, blood cholesterol level reduction, symptom free days<br>**CE Ratio** = Cost of treatment / Therapeutic effect<br>**Incremental ratio** = Difference in cost of treatment / Difference in therapeutic effect |
| Interpretation | "Most bang for your buck" Cost per life year gained Cost per patient cured, Cost per life saved, etc. |

# Cost utility analysis (CUA)

| When to use | Similar to CEA with the added dimension of a particular point of view, most often the patient. Intervention outcomes /consequence is measured in terms of patient preference (e.g. quantity and quality of life). |
|---|---|
| Application | CUA should be used when QoL is the important outcome:<br>• Psychological well-being, physical and social function are important in the treatment of arthritis<br>• Chemotherapy may increase survival but decrease well-being, both quality and quantity of life are important |
| Cost unit:<br>Outcomes unit:<br><br><br><br>Calculation | Monetary value<br>Health benefits across therapies are valued in similar units based on individual preferences<br>Utility values (QALY = years in health state *utility)<br>Cost per QALY gained or Cost per HYE gained |
| Interpretation | Lowest cost per unit QALY gained |

# Cost benefit analysis (CBA)

| When to use | For resource allocation decisions – ascertain whether the beneficial outcomes justify the cost. Comparison of different programs with different outcomes. |
|---|---|
| Application | Scope of analysis is usually broad, addressing large societal issues.<br>• AIDS prevention and awareness programs<br>• Smoking cessation intervention<br>• Diabetes drug adherence<br>• Breast cancer screening |
| Cost unit:<br>Outcomes unit:<br><br><br><br>Calculation | Monetary value<br>Measured in similar/different units and are always valued in monetary units (e.g. amount willing to pay to prevent a death, amount willing to pay to reduce exposure to a hazard)<br>Benefit to Cost ratio = Benefit ($)/Cost ($)<br>Net Benefit = Benefit ($) – Cost ($) |
| Interpretation | Choose alternative with the largest value (i.e. benefit) |

# Quasi-health economic evaluations

| Method | When to use |
|---|---|
| Cost-consequence | Where studies consider multiple outcomes, costs and benefits presented in a disaggregated form (e.g. health profiles). |
| Cost-of-illness (COI) | Evaluation of overall economic impact of a disease on a population.<br>Study that identifies and evaluates the direct, and sometimes indirect, costs of a particular disease or risk factor (e.g. smoking, alcohol consumption). |

# Summary of costs & outcomes for methods

| Method | Cost Unit | Outcomes measure | Interpretation of result |
|---|---|---|---|
| CMA | Monetary | Natural units (equivalent) | Choose option with lowest cost |
| CEA | Monetary | Natural clinical units (life-years gained, mmol/l blood glucose, mm Hg blood pressure) | Lowest cost per unit of effectiveness Incremental cost |
| CUA | Monetary | Quality adjusted life years (QALYs) or other utilities | Lowest cost per unit QALY |
| CBA | Monetary | Monetary | Ratios of greater than 1.0 |
| COI | Monetary | Monetary | Burden of disease / budget impact |

# Steps in conducting a pharmacoeconomic analysis

1) Define the pharmacoeconomic problem
2) Identify the study perspective
3) Identify the alternative interventions
4) Select the appropriate pharmacoeconomic method
5) Select primary data source and design
6) Select analysis techniques
7) Identify and measure outcomes of the alternative interventions
8) Establish probability of outcome events
9) Use decision analysis
10) Value costs and effectiveness
11) Sensitivity analysis
12) Interpret and present results

# 1. Define the problem and state the objective

- **Problem or research question** must be clearly defined and measurable.
- **Specific objectives** for comparing the effectiveness of alternative interventions must be selected. The objective(s) must reflect the dimensions of the health problem(s) and be relevant to the alternative interventions that will be compared. The measure should also be sensitive to the actual differences likely to be encountered between interventions.

- E.g. Identify the disease state and what aspect you want to deal with, such as 'What is the most cost-effective method for controlling glucose in the treatment of type 2 diabetes?"

# 2. Identify the perspective

- Several possible perspectives in an economic analysis:
  - Social Perspective (Impact on Society),
  - Patient Perspective,
  - Payer (Health Insurance) Perspective, or,
  - Care Provider (e.g. Hospital) Perspective.
- The Panel on Cost-Effectiveness in Health and Medicine - the societal perspective for CEA studies.
- Pricing Committee of South Africa - third party payer (i.e. a funder) perspective.
- Policy maker - different perspectives, such as patients, caregivers, third party payer and healthcare provider (societal perspective).
- **Perspectives determine which costs, and outcomes to include in analysis.**

# 3. Identify the alternative interventions

- A well defined problem and objectives provide boundaries to the choice of alternatives. The narrower the problem and objectives, the fewer relevant alternatives there will be.
- Comparators must meet the following criteria:
  - Be recognised as relevant by the scientific community,
  - Be representative in terms of medical practices, and,
  - Be representative in terms of health care costs.
- NB: The comparator doesn't have to be a drug therapy.

# 4. Most appropriate pharmacoeconomic method

- Depends on the scope of the problem and objectives

| Method | When to use |
|---|---|
| CMA | Two or more alternative treatments with clinically equivalent outcomes. |
| CEA | Choice between competing alternatives |
| CUA | Intervention outcomes /consequence is measured in terms of patient preference (e.g. quantity and quality of life). |
| CBA | Comparison of different programs with different outcomes |
| Cost-consequence | Where studies consider multiple outcomes, costs and benefits presented in a disaggregated form (e.g. health profiles). |
| Cost-of-illness (COI) | Evaluation of overall economic impact of a disease on a population. |

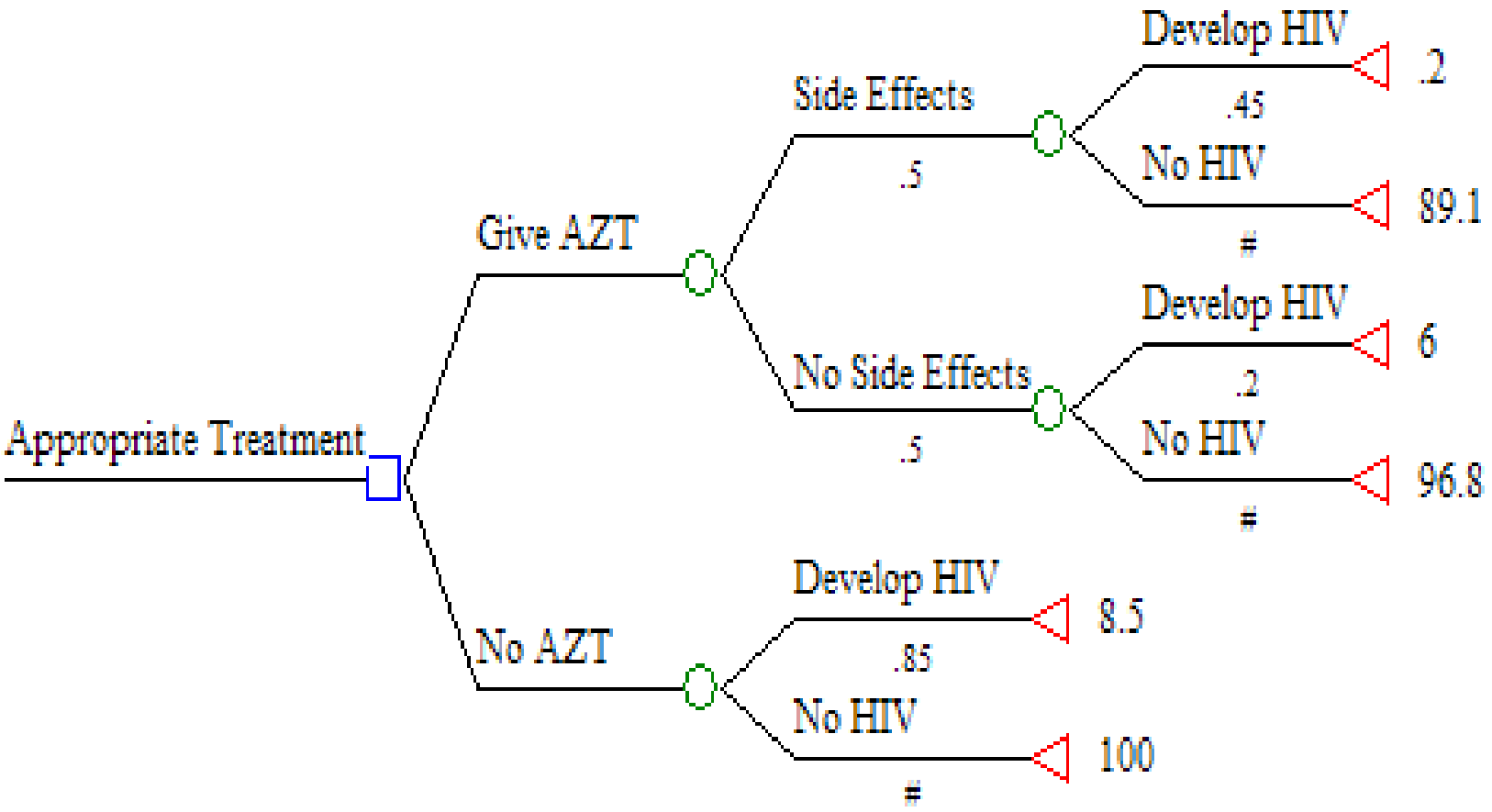
# 5. Select primary data source and design

- Retrospective analysis:
  - Clinical data from patients are too costly/not obtainable.
  - Gathering from past studies.
  - Comparisons and modelling provide a simulation of cost/effectiveness.
- Prospective analysis:
  - Obtaining clinical and economic data in a setting close to the "real" conditions.
  - Type I prospective analysis: add economic arm to clinical trial
- Clinical Trials
  - Randomised clinical trials
- Economic "Naturalistic" Trial
  - A comparative clinical protocol specifically designed to obtain data on effectiveness and economic aspects based on the same population in the same setting

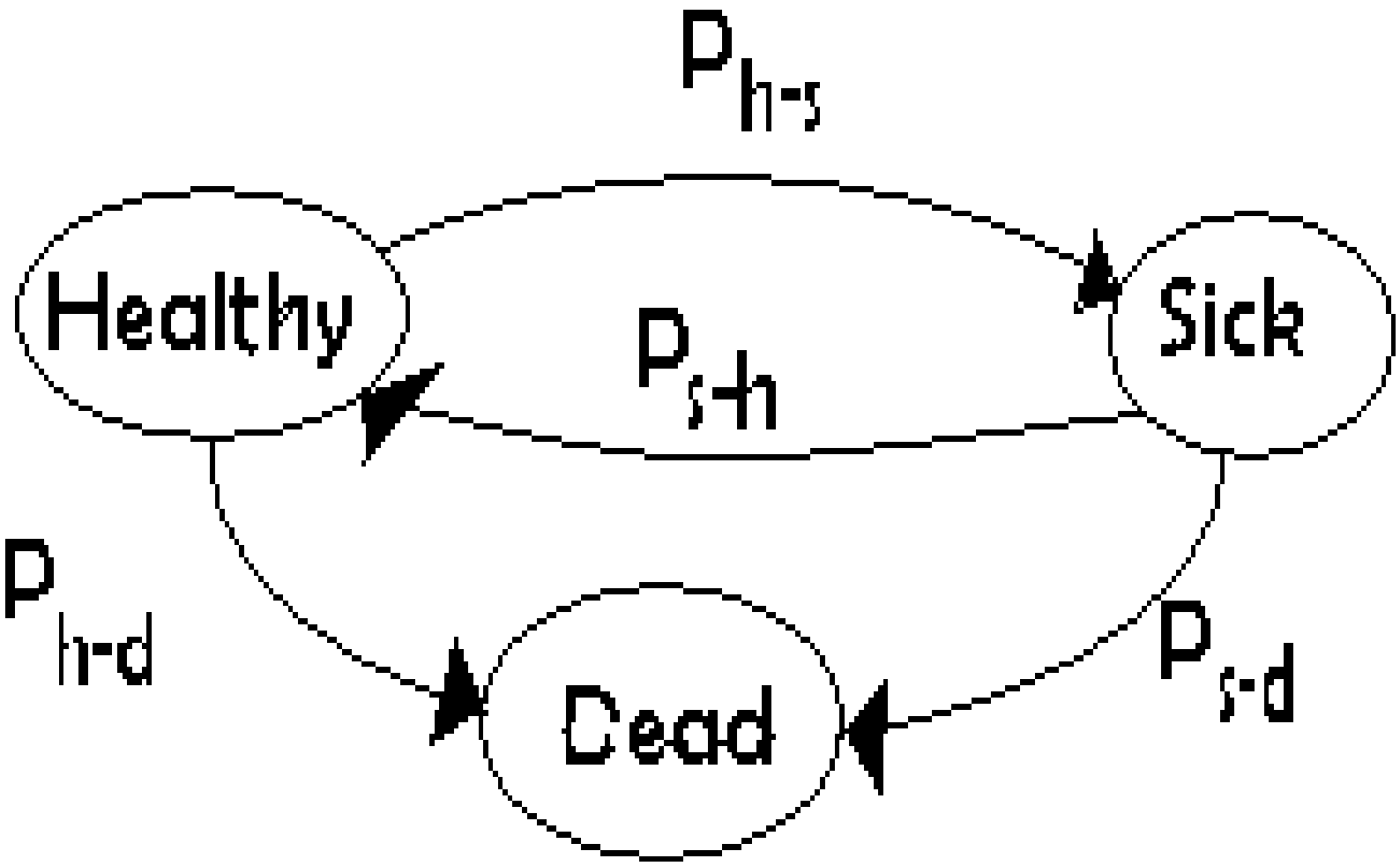
# 6. Select analysis techniques

- Modelling: Synthesises data from the literature to model the disease, treatment and outcome process.
  - Modelling option can make use of all available data from randomised clinical trials, observational studies, literature sources and meta-analysis, databases and expert opinion.
  - Usually a cost-effective or cost-utility ratio.
  - There are two types of models:
    - Decision Tree
    - Markov

# Decision Tree Models



# Markov models



# 7. Outcomes of the alternative interventions

- Health outcomes:
  - morbidity, mortality and quality of life
  - may occur as a result of both beneficial and adverse effects of an intervention. Probabilities of the various events that may result (e.g. cure, death, and adverse reactions) must also be established.
  - May take the form of final health output such as life-years gained, or an intermediate outcome measure such as patients appropriately treated.
- Resource outcomes:
  - Derived from both beneficial and adverse effects of an intervention. Beneficial effects result in resource savings and adverse effects in resource losses.
  - Both resource savings and resource losses can be either direct or indirect effects of the intervention.

# 8. Establish probability of outcome events

- The values of probabilities of health outcomes can be obtained by primary data collection from clinical literature or expert opinions, randomised clinical trials or observational studies.
- To derive a single probability estimate from multiple sources, meta-analysis can be used.

# 9. Use decision analysis

- Use of decision trees
- Decision tree displays the alternative clinical interventions being considered, the possible events (clinical, humanistic or economic) that may follow, their likelihood and cost, and the utility of the expected outcomes.
- It allows a quantitative consideration of beliefs about probabilities and preferences among possible outcomes.

# 10. Value costs and effectiveness

- Costs:
  - Costs are the actual amount of resources consumed to attain a certain benefit.
  - If the analysis spans more than a year, then the monetary values must be adjusted to a common point in time.
  - Discounting adjusts future costs or benefits using an expected interest or discount rate.
- Effectiveness (Benefits):
  - The unit in which effectiveness is valued is dependent on the type of pharmacoeconomic method used (cost-effectiveness, cost-benefit, cost-utility).

# 11. Sensitivity analysis

- Data used in pharmacoeconomic studies will always include some uncertainties and potential biases.
- "Sensitivity analysis" determines the degree to which this uncertainty could influence conclusions about the economic impact of clinical decisions.
- In sensitivity analysis, results are calculated separately for different values of particular elements of the study (e.g. cost of a particular treatment or epidemiological data regarding incidence or cure rates).

# 12. Interpret and present results

- A final report should be produced which should clearly describe methods, data sources, cost sources and assumptions. A model, if used, should be described, and supported by clinical trial data, literature, or epidemiological data.
- Methods for data collection should be clearly stated and justified with a description of validity and sources of verification.
- The particular variables selected for sensitivity analysis and the form of sensitivity analysis used should be described and justified so that readers can determine the robustness of the study results.
- Study limitations and caveats including significant omissions should be clearly stated in order to provide transparency.

# Pharmacoeconomics applications

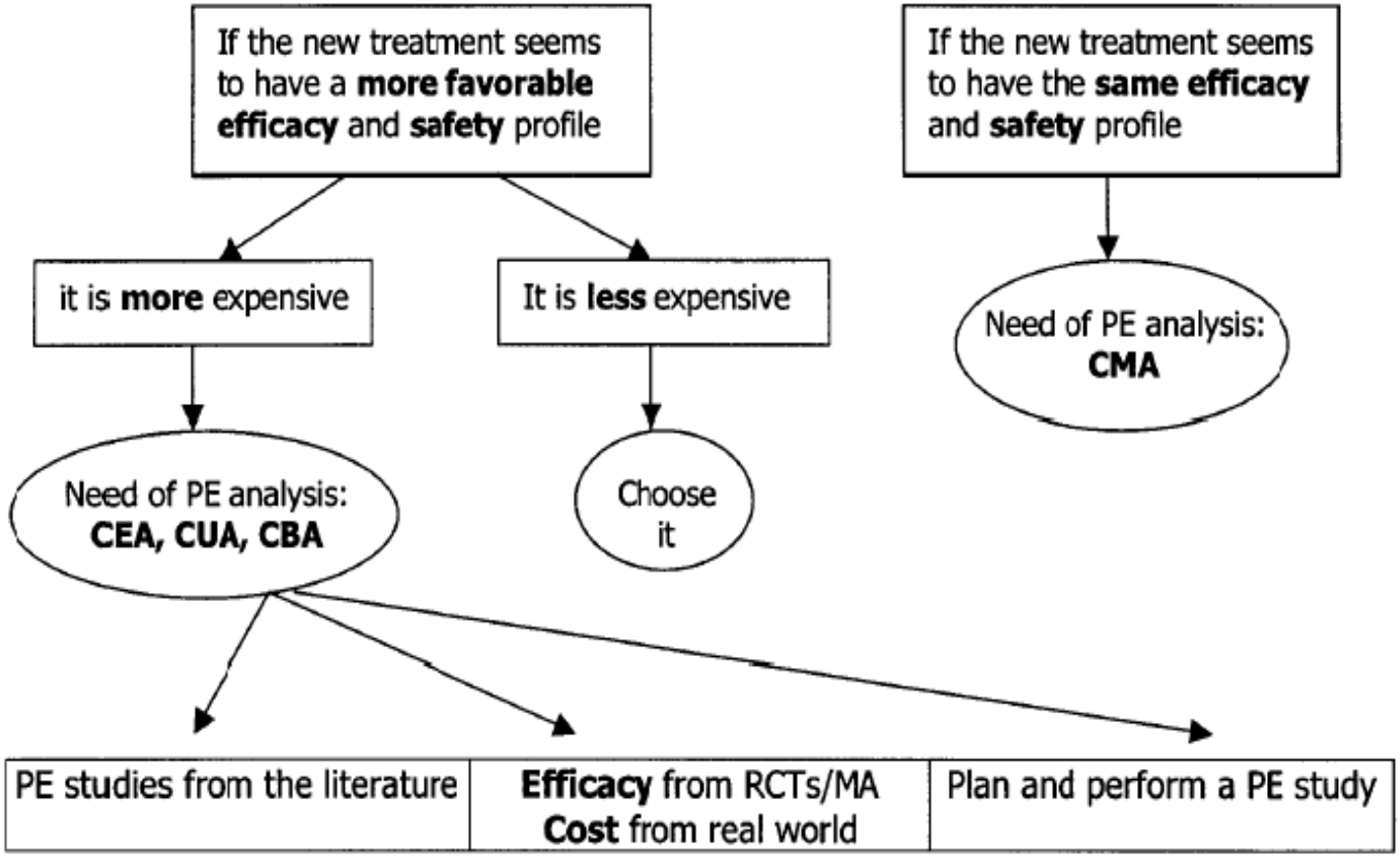
- Treatment guidelines: more efficient use of the resources available to treat the disease
- Decision-making in health care organisations:
  - Designing of the pharmacy benefit package,
  - Establishing contractual relationships and negotiating prices with individual pharmaceutical manufacturers,
  - Influencing the prescribing behaviour of physicians,
  - Influencing the use of pharmaceuticals by patients.
- Approval/Reimbursement decisions: ICER of a new drug be less than or equal to a specific predetermined level before approval
- Pricing decisions: defend prices of patented drugs/set prices so that the drug becomes cost-effective

# Pharmacoeconomics applications continued

- Research and development decisions: identify promising areas for research and development investment/ go/no go decisions at critical points in the drug's development
- Post-marketing surveillance: update decisions on pricing, formulary listings and clinical guidelines
- Marketing:
  - to make claims with regard to outcomes associated with products,
  - to demonstrate the cost-effectiveness of a product,
  - to illustrate to the buyer the cost associated with selected diseases for which drug therapy is an important component of treatment,
  - to demonstrate superiority of a particular product, and,
  - to justify a price that may be higher than that of generic competitors or substitute therapies.

# Pharmacoeconomics applications example

- Application of pharmacoeconomics to formulary decision making:




Legend: RCTs = (randomized controlled trials); MA = meta-analysis

# Thank you

It all starts here

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