Practical considerations - pharmacoeconomic guidelines

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Disclaimer

This presentation reflects my own views and does not reflect the views of the SA government or any other organization or committee that I have been affiliated with.

Outline

- Simple vs complex models
- Role of costing studies
- Link with health outcomes research
- Source of data
- PE evaluation and re-imbursement
- Interpretation of clinical trials
- Way forward

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Simple models vs complex modelling

Section 8 : Modelled Evaluation

‘The Pricing Committee discourages the use of complex models where a simple model will adequately support the economic argument. Only models that are transparent, as determined by the Pricing Committee will be considered’.

Transparency:

- structure of the model
- information required to test assumptions and inputs

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Simple vs complex models

Section 8.1

Prior to development of a model to support a pharmacoeconomic analysis, an application for the use of a model must be submitted in writing to DPEE with the following information:

- South African approved brand name, INN and MCC registration number of the medicine;
- Key clinical trials intended for use in the model (e.g. extrapolation of survival data);
- Justification for use of that particular model;
- Type of model;
- Description of design of model including schematic diagrams;
- Main clinical outcome to be modelled;
- Time horizon for the model; and
- How the model intends to handle uncertainty (i.e. probabilistic sensitivity analysis)

Simple vs complex models

Section 8.2 – Modelled Options

- Identify the options considered and justify the option
- Consider implicit assumptions built into model structures and comment

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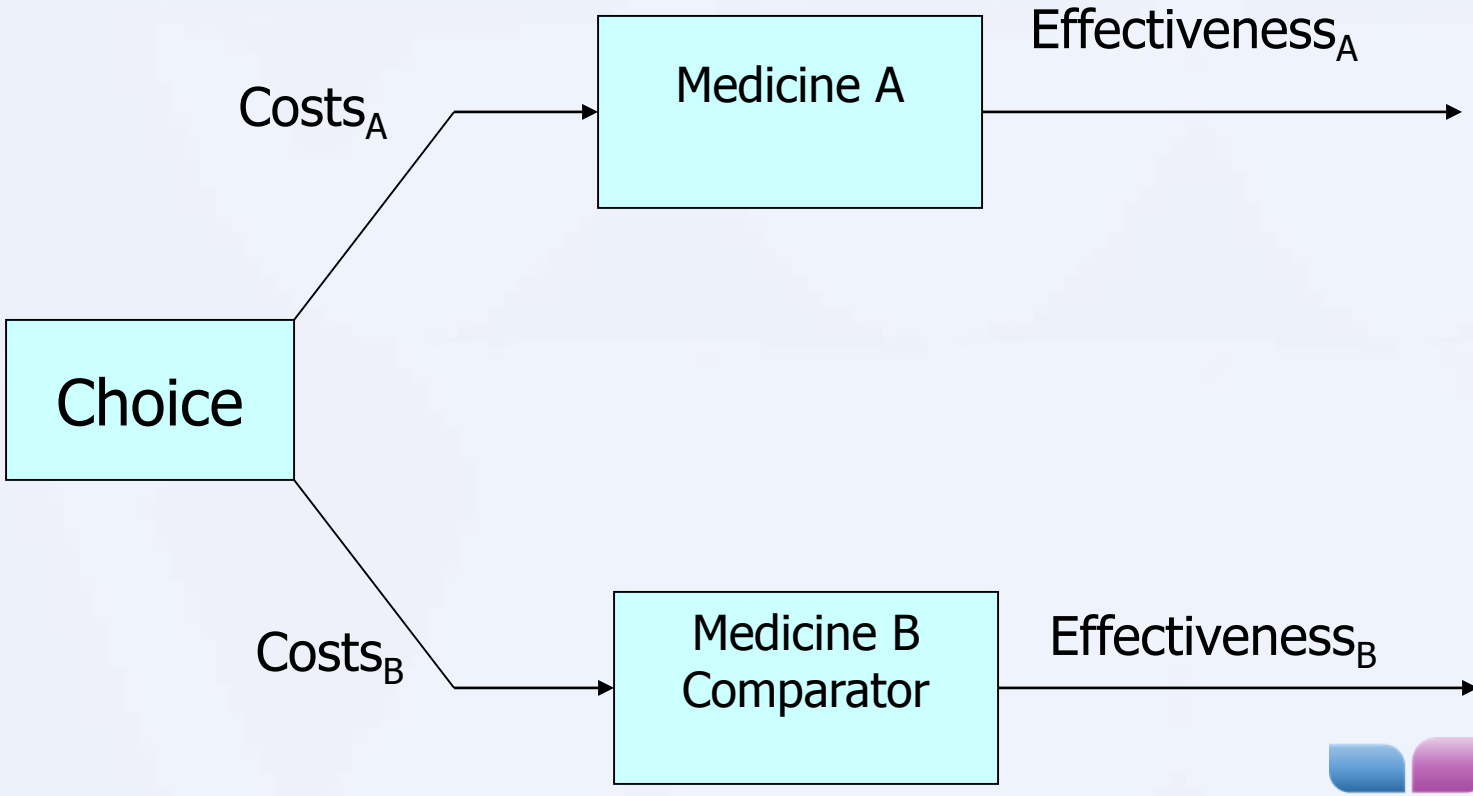
Pharmacoeconomic Evaluation



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Simple models

Value of simple model

- Clear
- Transparent – understand workings, re-run
- Certainty of result – follow-through
- Confidence in decision making – correctness

Complex models

Experience with Non-linear mixed-effects modelling (NONMEM)

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$$Cp_{ss} = -\frac{1}{2}\left[\left(\frac{Vm}{Cl} + Km - \frac{R}{Cl}\right) - \sqrt{\left(\frac{Vm}{Cl} + Km - \frac{R}{Cl}\right)^2 + \frac{4.R.Km}{Cl}}\right]$$

$$\text{Vm} = (\theta_1 * WT * \theta_3)\ \text{RACE* SMK * ALC * SEX * AGE * EXP}n_1$$

Where RACE = θ_4 if coloured, otherwise = 1
SMK = θ_5 if smoker, otherwise = 1
ALC = θ_8 if drinker, otherwise =1
SEX = θ_9 if male, otherwise = 1
AGE = θ_{10} if ≥ 65 years, otherwise = 1

$$Km = \theta_2 * \text{RACE} * \text{AGE} * \text{EXP}n_2$$

where RACE = θ_7 if coloured, otherwise = 1
AGE = θ_{11} if ≥ 65 years, otherwise = 1

$$Cl = \theta_{11} * \text{EXP}n_3$$

P. Valodia et al. Factors influencing the population pharmacokinetic parameters of phenytoin using non-linear mixed effects modelling in adult epileptic patients in South Africa. Therapeutic Drug Monitoring. 1999.21: 57-62

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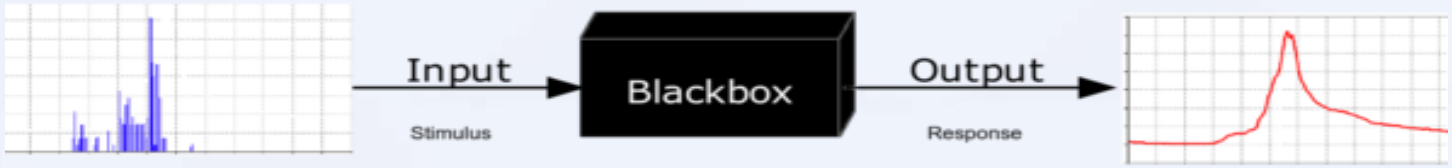
Black box effect

- In science, computing, and engineering, a black box is a device, system or object which can be viewed in terms of its inputs and outputs (or transfer characteristics), without any knowledge of its internal workings. Its implementation is "opaque" (black).



- The usual representation of this black box system is a data flow diagram centred in the box.
- The opposite of a black box is a system where the inner components or logic are available for inspection.

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Complex models

- Markov modelling (can also be a simple model)
- Follow through the workings of the model
- Still make a judgement decision

Role of costing studies

- Minimal cost data
- Actual costs associated with an episode of care
- Sharing of cost data
- Simulations
- Time motions study – public sector

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Link between PE and outcomes research

Definition: Health Outcomes

A **scientific** discipline that evaluates the effect of **health care interventions** on patient-related, if not **patient specific**, economic, clinical and humanistic outcomes.

Outcomes research is generally based on the conceptual framework that evaluation of treatment alternatives involves simultaneous assessment of multiple types of outcomes that are disease-related.

ISPOR BOOK OF TERMS, 2003

- Real-world evidence related to effectiveness
- More focus on health outcomes
- Challenging area

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- ‘Outcome measurement is perhaps the single most powerful tool in revamping the health care system’

- ‘Measure outcomes for every patient’

Michael Porter

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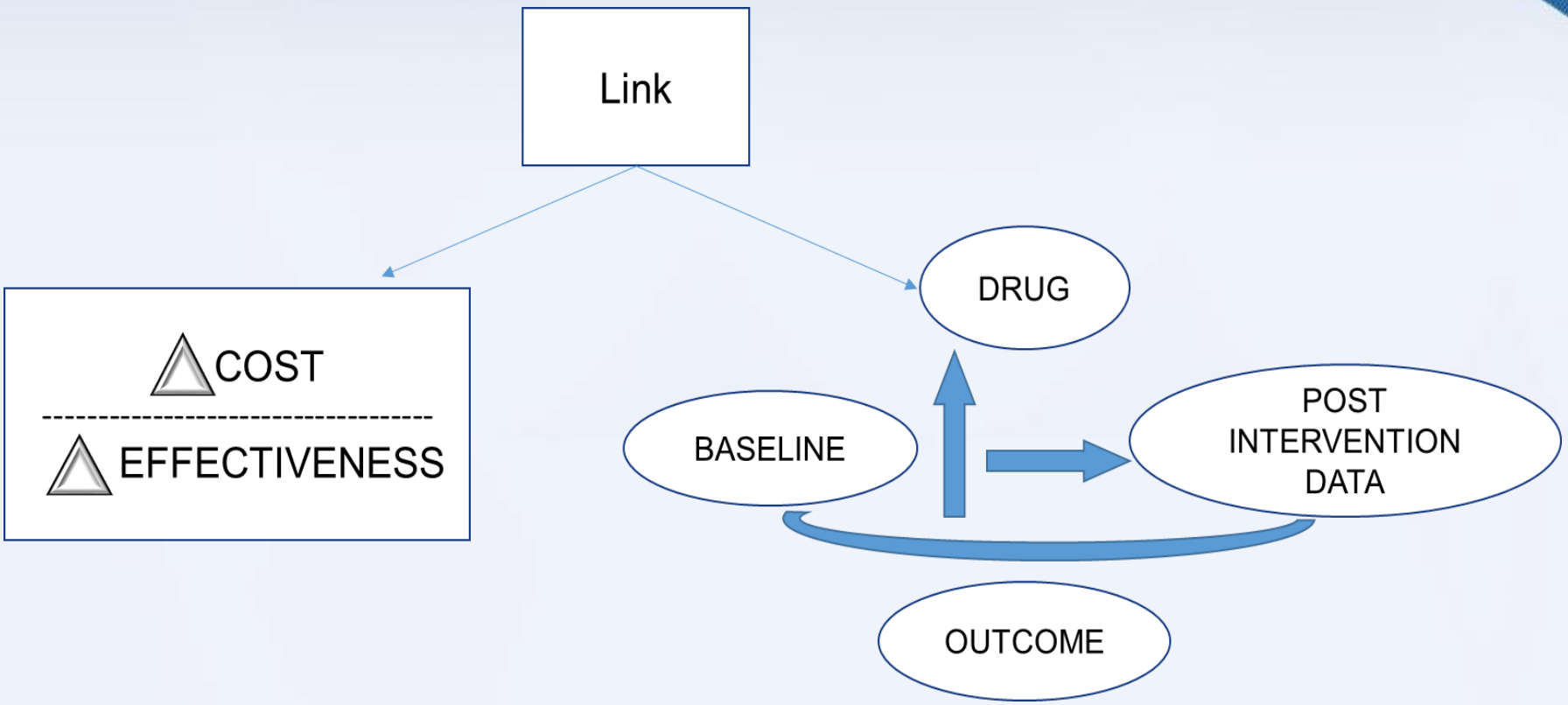

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Source of data

- Cost data
- Effectiveness data
- Managed care companies
- Pay for data – governance and transparency
- Win-win situation
- Database of costs – private and public sector

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PE evaluation and re-imbursement

- Are these phenomena integrally linked?
- Can government decide how medical schemes should spend their money?
- Where do budget impact analyses fit in?
- The approach differs for public and private sectors

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Interpretation of clinical trials

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Multiple Sclerosis and Related Disorders (2015) 4, 187–191

Available online at www.sciencedirect.com

ScienceDirect

journal homepage: www.elsevier.com/locate/msard

REVIEW

Smoke and mirrors: Limited value of relative risk reductions for assessing the benefits of disease-modifying therapies for multiple sclerosis

Magd Zakaria*

Faculty of Medicine, Ain Shams University, 44 ElAhram street, Heliopolis, Cairo, Egypt

Received 29 January 2015; received in revised form 29 March 2015; accepted 31 March 2015

	Trial (1) Low	Trial (2) Inter	Trial (3) High
Risk (relapse) in Placebo	4%	40%	90%
Risk (relapse) in new medicine	2%	20%	60%
Relative Risk Reduction (RRR)	50%	50%	33%
NNT	50	5	3
Absolute Risk Reduction (ARR)	2%	20%	30%

A high RRR may result from a *clinically insignificant* change in the event rate if the event rate of the placebo is very low (trial 1)

Magd Zakaria, Multiple Scelerois and Related Disorders (2015),4,187-191

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Summary

- The model and is workings should be clear
- Need to conduct more costing studies
- Conduct outcomes research
- Need to establish sources for data
- Share data
- Ensure proper interpretation of clinical trials

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Way forward

- Mandatory submissions
- Inform policy decisions
- Review content of guidelines
- Understand the critical success factors
- Comprehensive submissions
- Sponsorships for training

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