

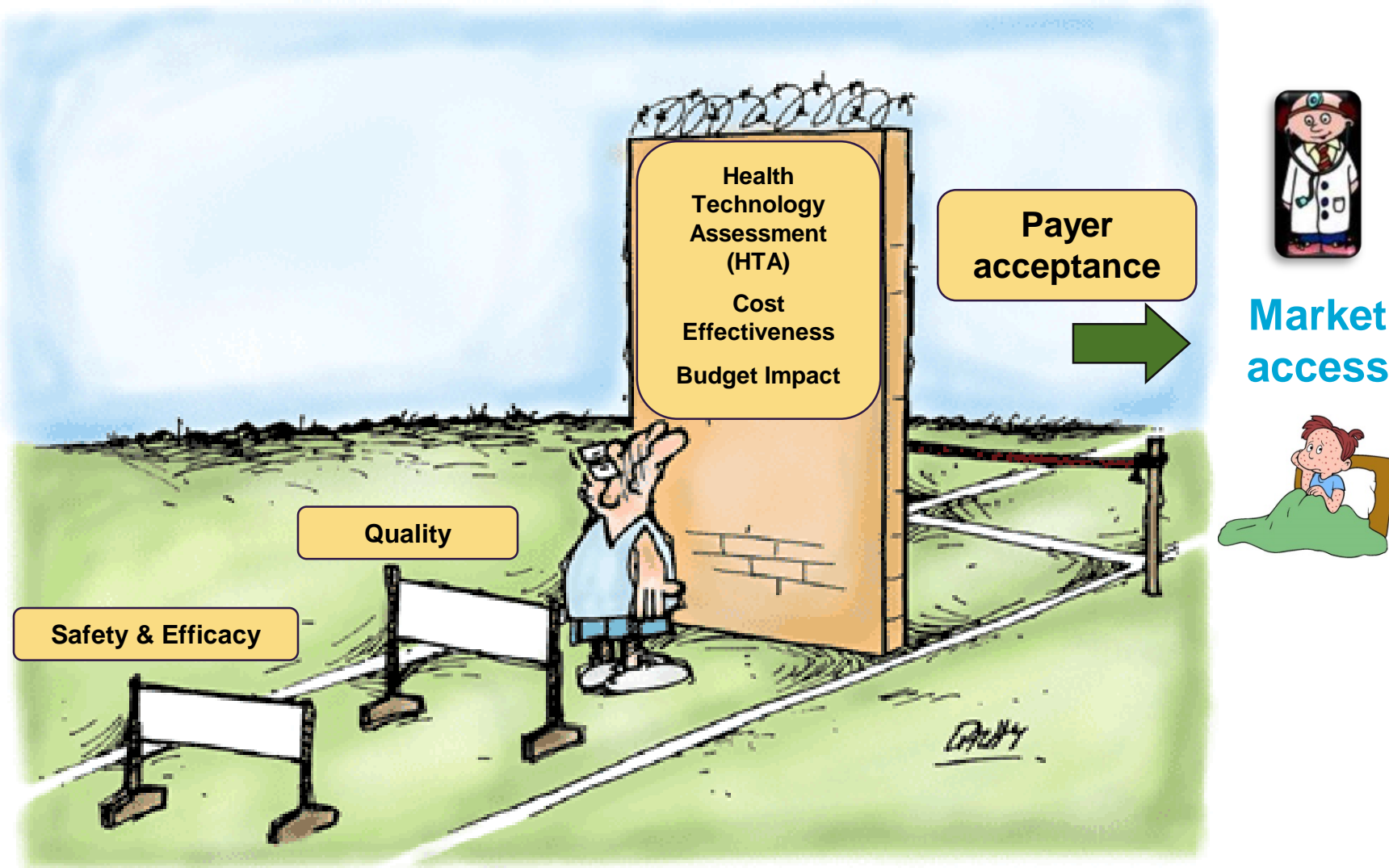
# The Role of Health Economics and Pharmacometrics for Cost-effective Patient Care



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2018 ASCPT Preconference: Pharmacometrics Meets Health Economics  
March 21, 2018

# Economic Evaluation & Market Access



# Evidence Requirements & Pharmacoeconomic Guidelines



## COUNTRY-SPECIFIC PHARMACOECONOMIC GUIDELINES

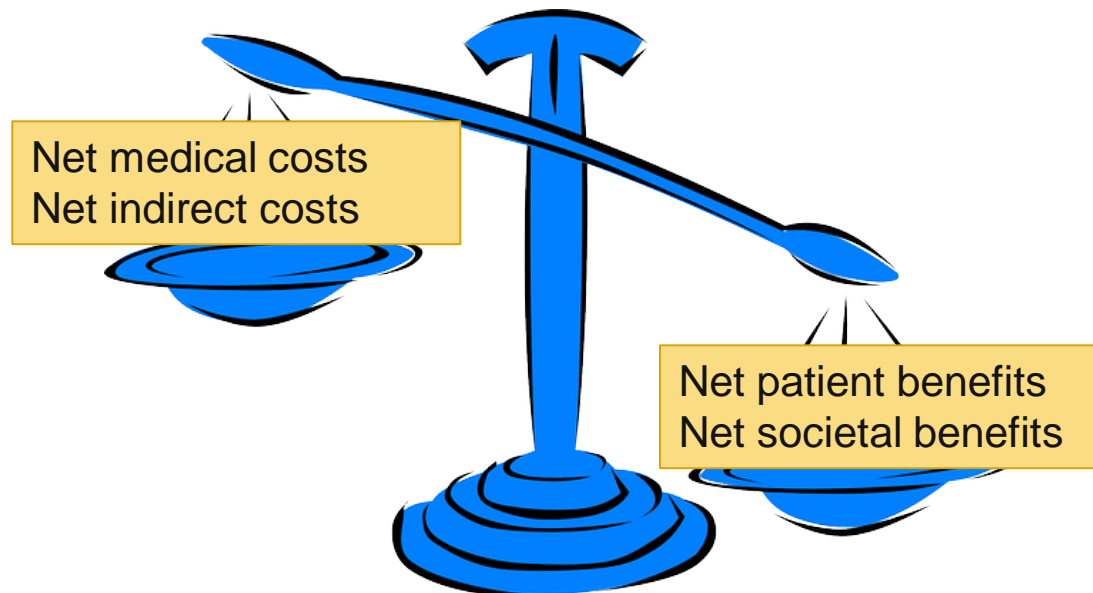
	Published PE Recommendations	PE Guidelines	Submission Guidelines
Africa	<a href="#">South Africa</a>	<a href="#">Egypt</a>	
America-Latin		<a href="#">Brazil</a> <a href="#">Colombia</a> <a href="#">Cuba</a> <a href="#">México</a> <a href="#">MERCOSUR (Argentina, Brazil, Paraguay, Uruguay)</a>	
America-North	<a href="#">United States</a>	<a href="#">Canada</a>	
Asia	<a href="#">China Mainland</a>	<a href="#">Japan</a> <a href="#">Malaysia</a> <a href="#">Taiwan</a> <a href="#">South Korea</a>	<a href="#">Israel</a> <a href="#">Thailand</a>
Europe	<a href="#">Austria</a> <a href="#">Denmark</a> <a href="#">Hungary</a> <a href="#">Italy</a> <a href="#">Russian Federation</a> <a href="#">Spain</a> <a href="#">Croatia</a>	<a href="#">Baltic (Latvia, Lithuania, Estonia)</a> <a href="#">Belgium</a> <a href="#">France</a> <a href="#">Germany</a> <a href="#">Ireland</a> <a href="#">The Netherlands</a> <a href="#">Norway</a> <a href="#">Portugal</a> <a href="#">Slovak Republic</a> <a href="#">Slovenia</a> <a href="#">Sweden</a> <a href="#">Switzerland</a>	<a href="#">Czech Republic</a> <a href="#">England &amp; Wales</a> <a href="#">Finland</a> <a href="#">Poland</a> <a href="#">Scotland</a> <a href="#">Spain - Catalonia Region</a>
Oceania		<a href="#">New Zealand</a>	<a href="#">Australia</a>

# Health Economic Evaluation

## Core Question

Is this health procedure, service, or programme worth doing compared with other things we could do with these same resources?

(Drummond et al., 1987)



# Basic Elements of Measuring Value

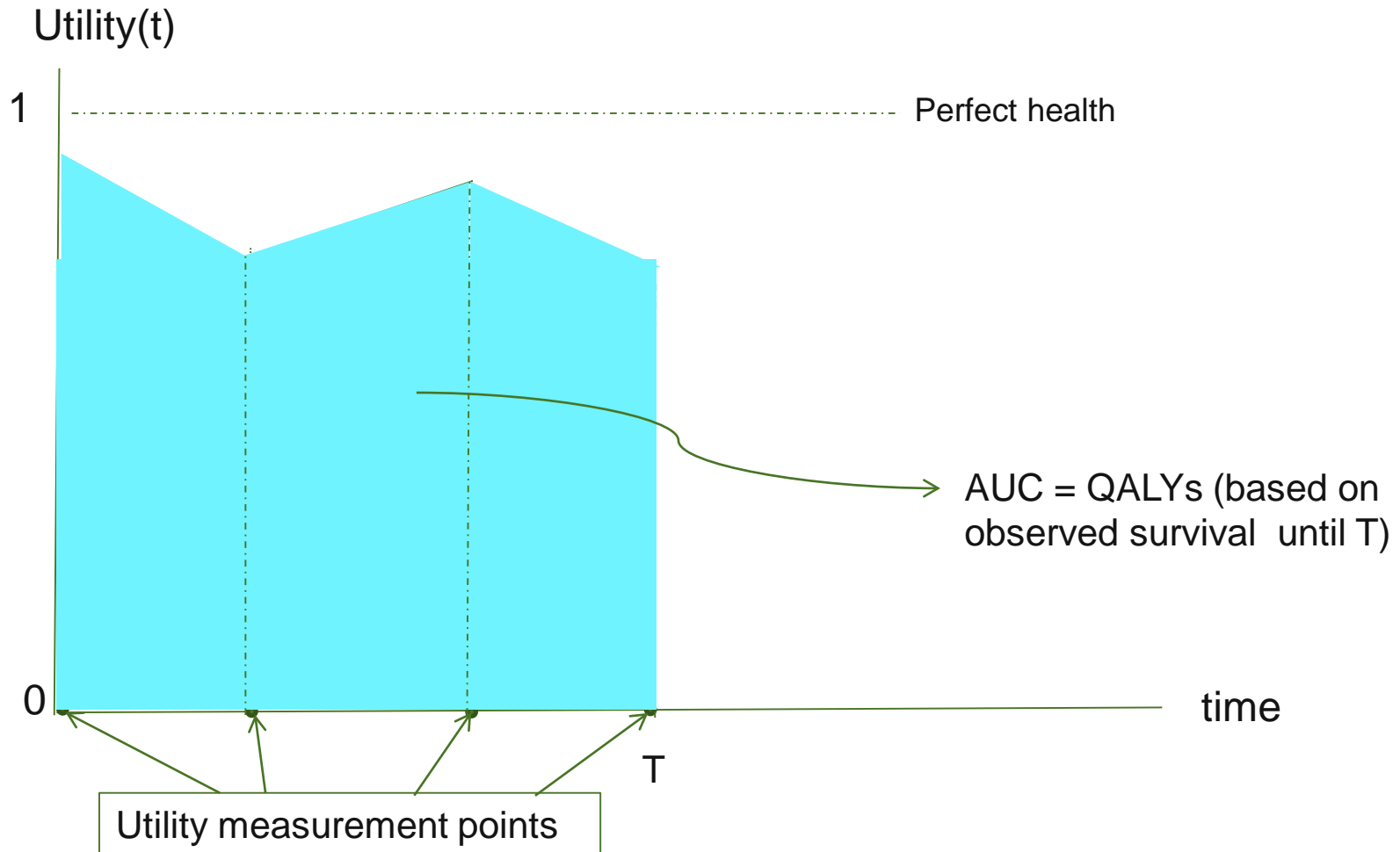
**Cost:** What is the net additional cost when the new treatment is used instead of another one?

**Benefit:** What's the net health benefit from the new treatment, compared to others?

**Cost-benefit (aka cost-effectiveness) ratio:**

What's the cost per additional unit of health? Is the patient or society willing to pay that much for the new treatment?

# Quality-Adjusted Life-Year (QALY) Concept Review



# Incremental Cost-Effectiveness Ratio (ICER)



$$\text{ICER (for drug T vs drug C)} = \Delta C / \Delta E$$

Where:

$\Delta C$  = Additional total cost of drug T vs drug C

= drug cost difference + resource use cost difference

$\Delta E$  = Additional effectiveness of drug T vs drug C

Example:

$$\Delta C = \$5000$$

$\Delta E = 0.2$  Quality Adjusted Life Years (QALYs)

$$\text{ICER} = \$5000/0.2 = \$25,000 \text{ per QALY saved}$$

# ICERs and Net Monetary Benefit

Decision rule for adoption (based on econ evaluation alone):

→ Adopt if cost per QALY saved due to new treatment is less than society's willingness to pay ( $\lambda$ ) for a QALY

→ i.e., if  $\Delta C / \Delta E < \lambda$

if  $\Delta C < \lambda \Delta E$

if  $0 < \lambda \Delta E - \Delta C$

$\lambda \Delta E - \Delta C$  is known as the “net monetary benefit (NMB)”

so we adopt if  $NMB > 0$

Let's say  $\lambda = \$100,000$  per QALY saved, and  $\Delta E = 0.2$ .

Then  $\lambda \Delta E = \$20,000$ ; if  $\Delta C = \$5,000$ , then  $NMB = \$15,000$ .



# One country's view on cost-effectiveness

## NICE Cost-effectiveness Principles

<£20k per QALY

- **Cost effective**



**Majority of drug recommendations for unrestricted use**

£20-£30k per QALY

- **Borderline cost-effective,**
- **limit guidance to patients which are**



**Typically recommended for restricted use**

>£30k per QALY

- **Generally not cost-effective**



**Recommend for restricted use or not recommended**

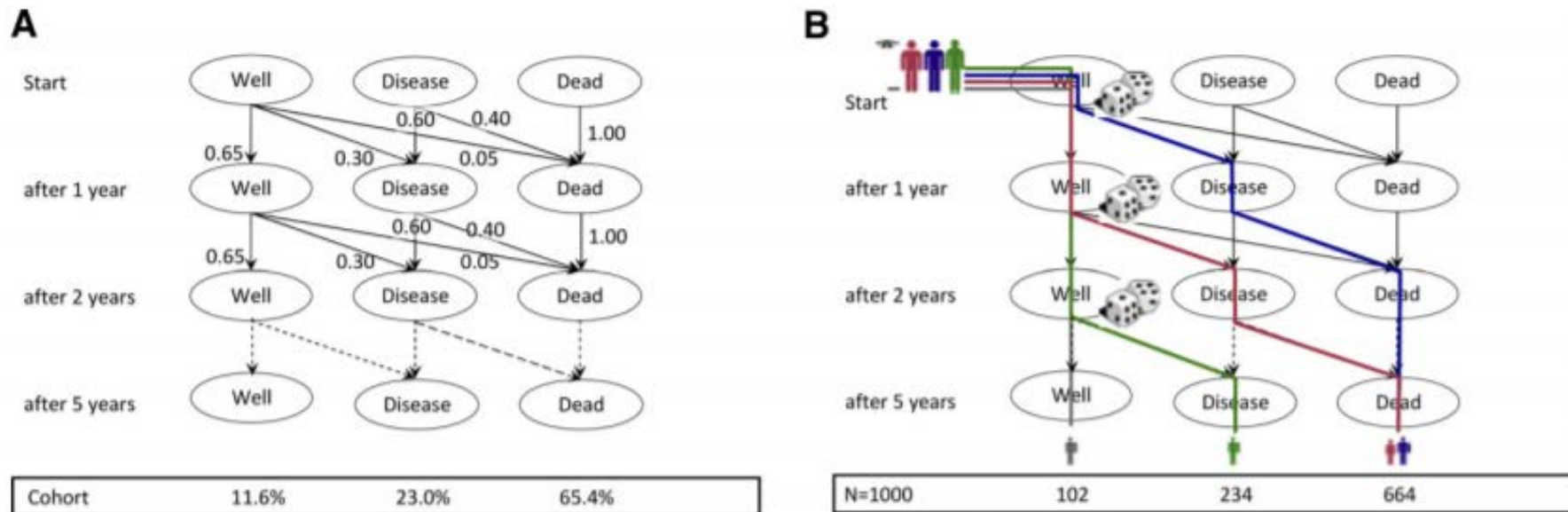
“It is apparent that the appraisal committee has been reluctant to recommend the use of technologies with a cost effectiveness ratio of more than £ 30,000 [per QALY gained].”

*Michael Rawlings, Chairman NICE, cited in SCRIP*

# Early economic models

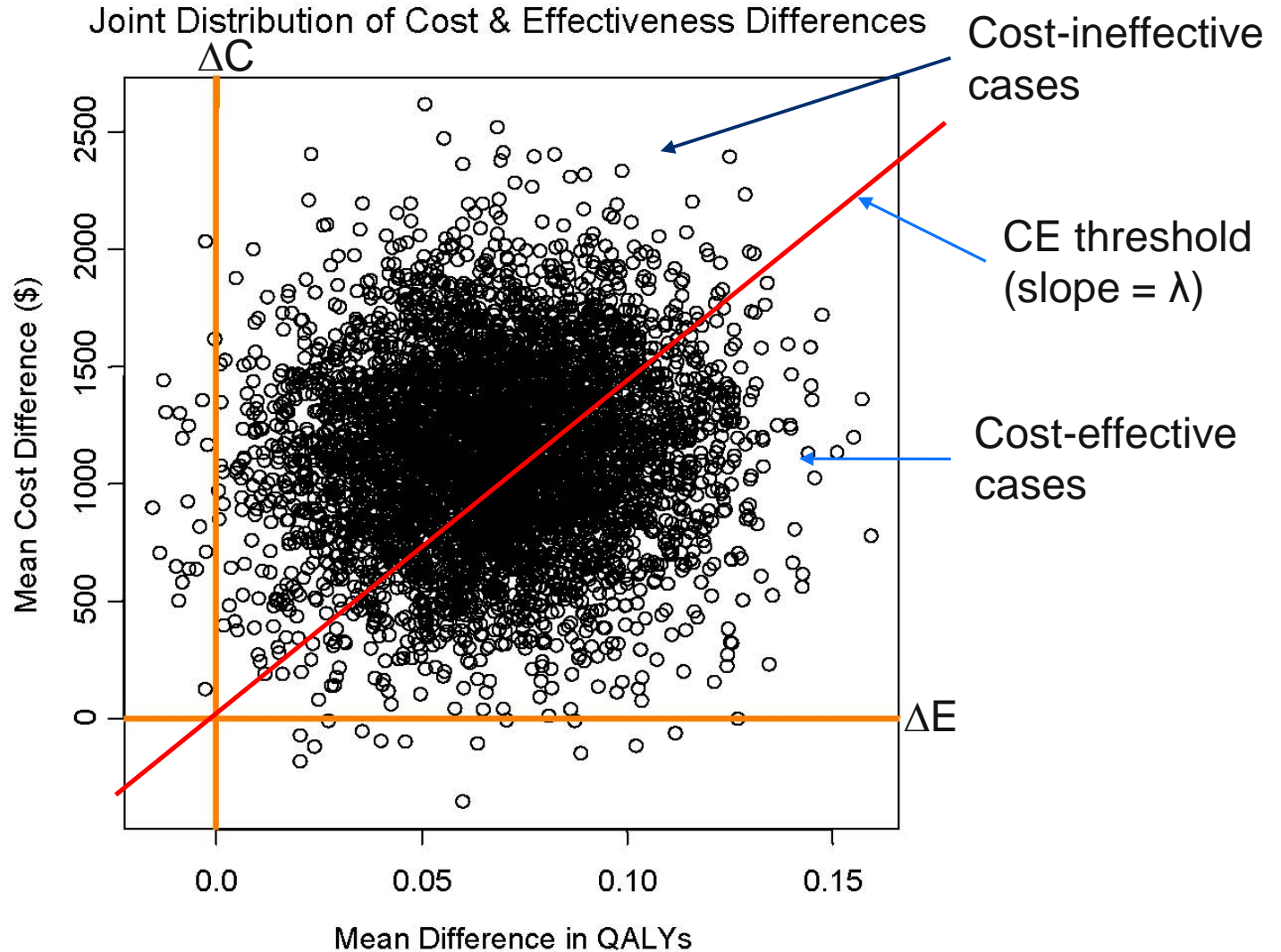
- Typically disease-based models for early product teams, in phase 2 or earlier
- Captures basic disease treatment patterns, outcomes, and costs
- Allows for variations in treatment prices and outcomes, and calculates the cost-effectiveness of treatment
  - Estimates the product price that will be consistent with cost-effectiveness for **an expected treatment effect**
  - Or, estimates the treatment effect necessary to support a given product price, and stay within a cost-effective range
- Primarily meant for internal company use

# A common type of health economic model – The state-transition model

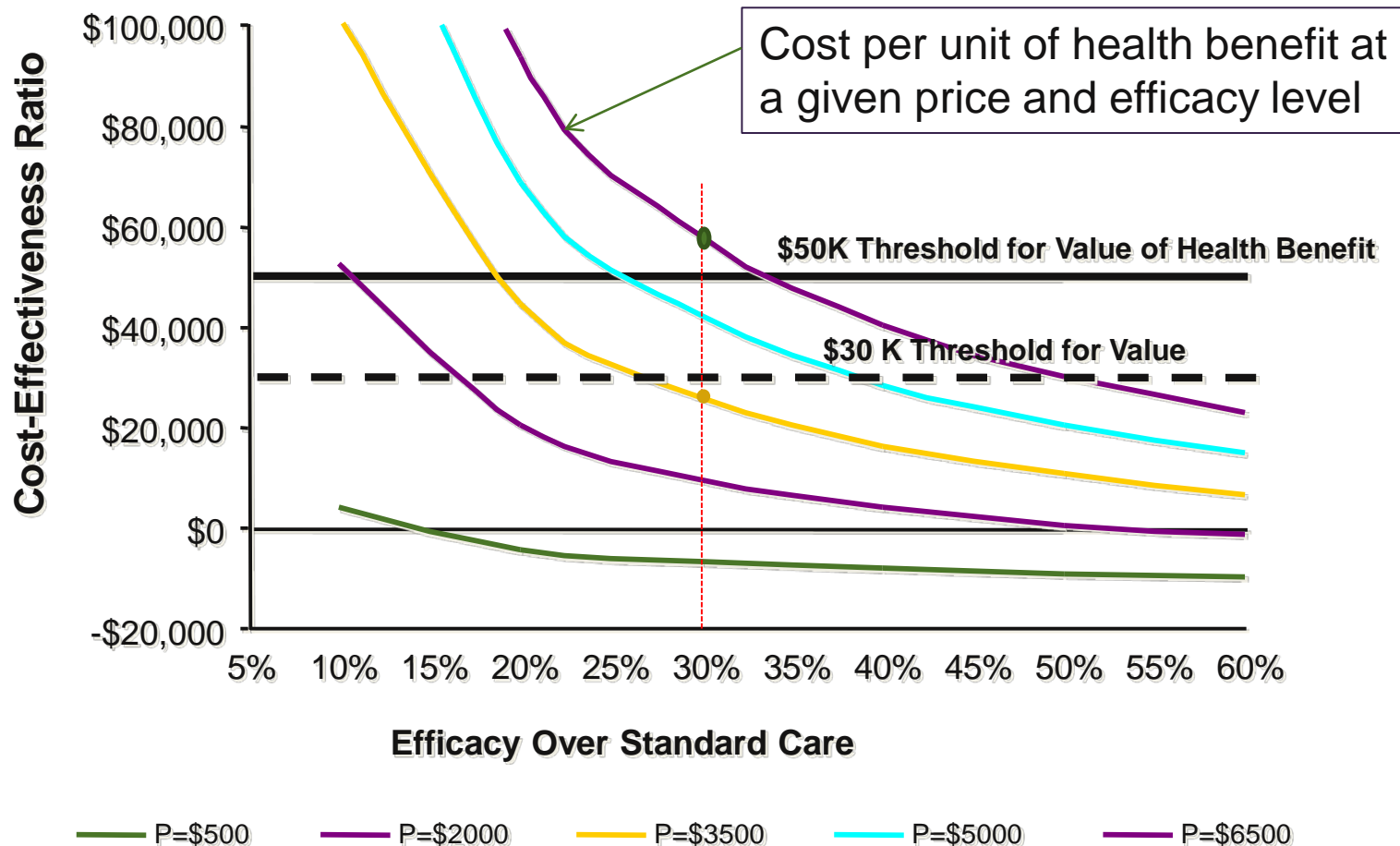


**Fig. 1 – In a cohort simulation (A), the entire cohort is (re-)distributed across states after each cycle. In an individual-level microsimulation (B), a finite number of individuals are simulated by using first-order Monte Carlo microsimulation. In this simple example, all individuals start in the state ‘Well’ and the disease is chronic (i.e., there is no regression from “Disease” to “Well”). In principle, individuals can start in different states and they can regress to states they have already been in. (A) Cohort simulation in a state-transition model. (B) Monte Carlo simulation in a state-transition model.**

# Cost-effectiveness results from stochastic models



# How Well Does The Target Outcome Profile Measure Up?

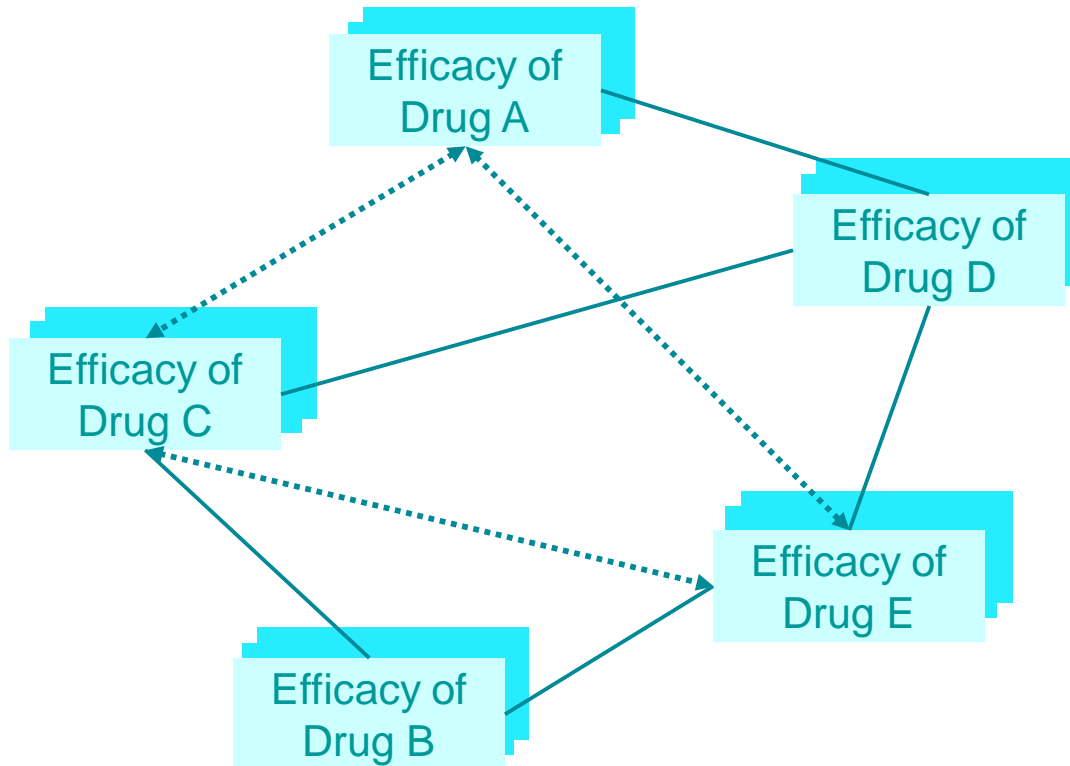


# Mixed/indirect treatment comparisons



- Provides treatment comparisons when head-to-head trial data are not available
- For some outcomes, given sufficient data, pharmacometric models can provide this evidence
- More often is done using pairwise comparisons taken from the literature, using network meta-analysis (now typically Bayesian) or model-based techniques
- Can be used as evidence of comparative effectiveness per se, or as input for economic models

# Mixed treatment comparisons





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## SCIENTIFIC REPORT

# Interpreting Indirect Treatment Comparisons and Network Meta-Analysis for Health-Care Decision Making: Report of the ISPOR Task Force on Indirect Treatment Comparisons Good Research Practices: Part 1

Jeroen P. Jansen, PhD<sup>1,\*</sup>, Rachael Fleurence, PhD<sup>2</sup>, Beth Devine, PharmD, MBA, PhD<sup>3</sup>, Robbin Itzler, PhD<sup>4</sup>, Annabel Barrett, BSc<sup>5</sup>, Neil Hawkins, PhD<sup>6</sup>, Karen Lee, MA<sup>7</sup>, Cornelis Boersma, PhD, MSc<sup>8</sup>, Lieven Annemans, PhD<sup>9</sup>, Joseph C. Cappelleri, PhD, MPH<sup>10</sup>

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## ISPOR TASK FORCE REPORTS

# Modeling Good Research Practices—Overview: A Report of the ISPOR-SMDM Modeling Good Research Practices Task Force-1

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