

Recommendations of the Second Panel on Cost-Effectiveness in Health and Medicine

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Stanford University

**Acknowledgment: Gillian Sanders, Peter Neumann and
the 2nd Panel**

Recommendations of the
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Cost-Effectiveness Analysis

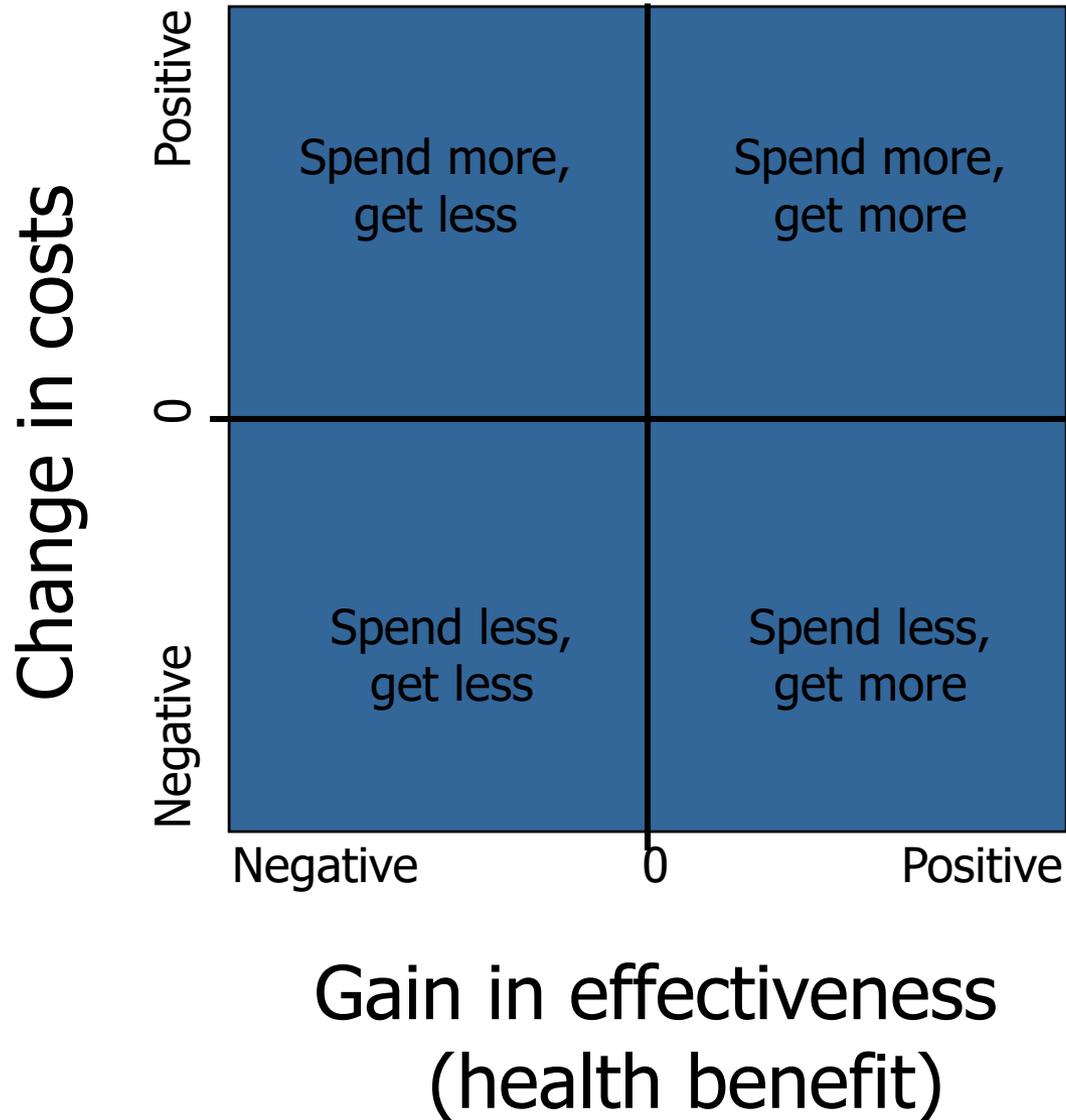
- Goal of cost-effectiveness analysis is to assess the value of health care interventions
- Value: do benefits justify costs?
 - Is HIV screening cost effective?
 - Are implantable defibrillators cost effective?
 - Is care coordination after admission for heart failure cost effective?

CEA

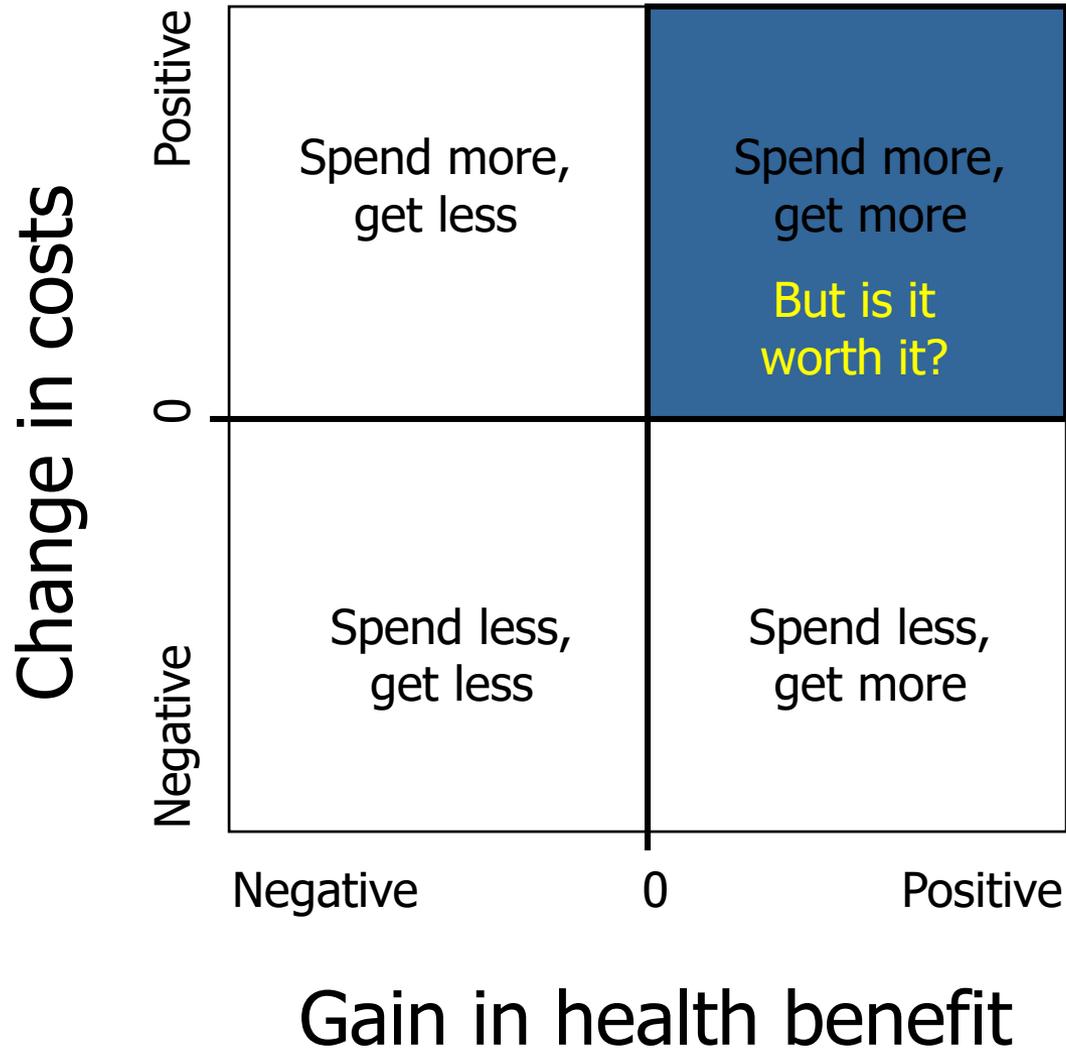
- Compares two or more strategies
 - E.g., screening to no screening
- Assesses the incremental benefit and incremental cost of one strategy versus another
- Calculates the incremental cost-effectiveness ratio:

$$\frac{\text{Costs with screening} - \text{Costs without screening}}{\text{Benefits with screening} - \text{Benefits without screening}}$$

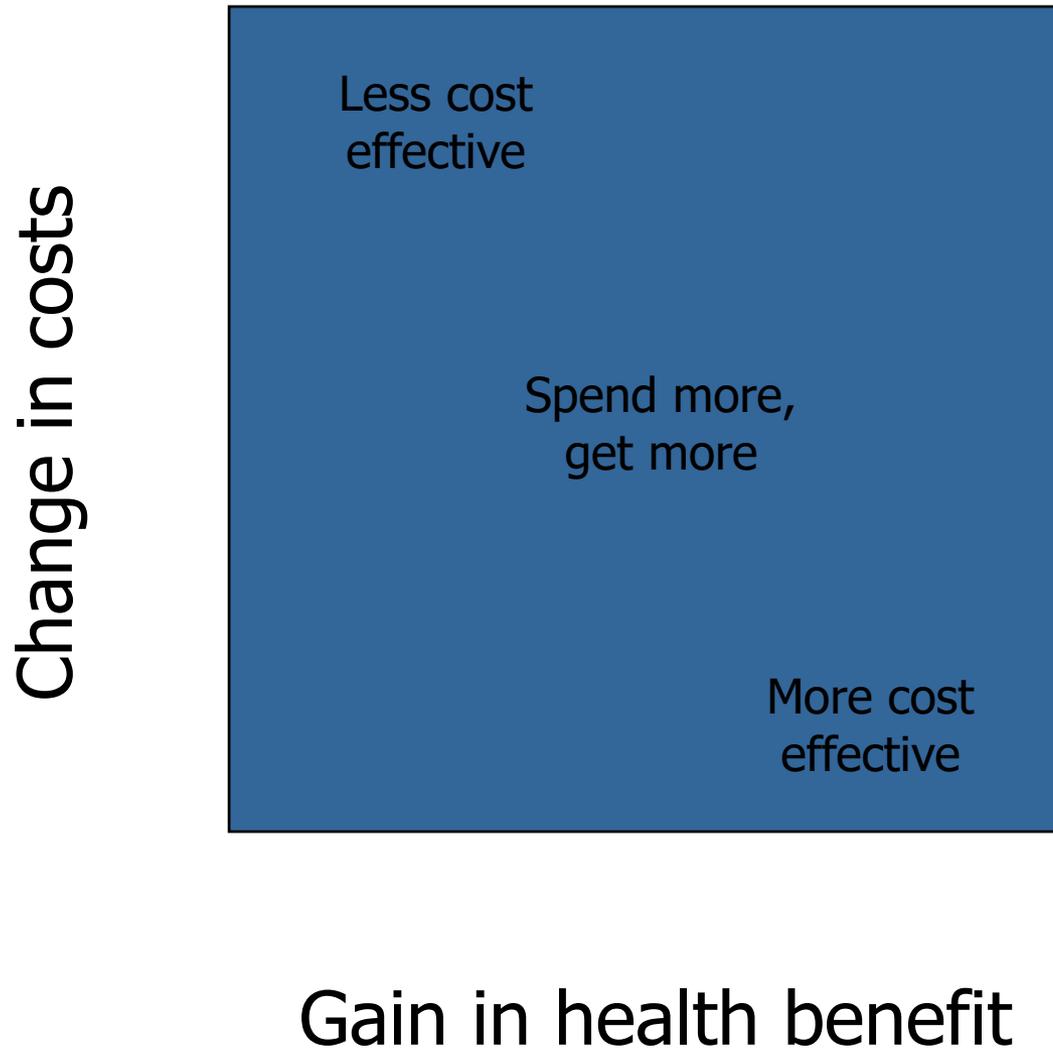
Accounting for Value



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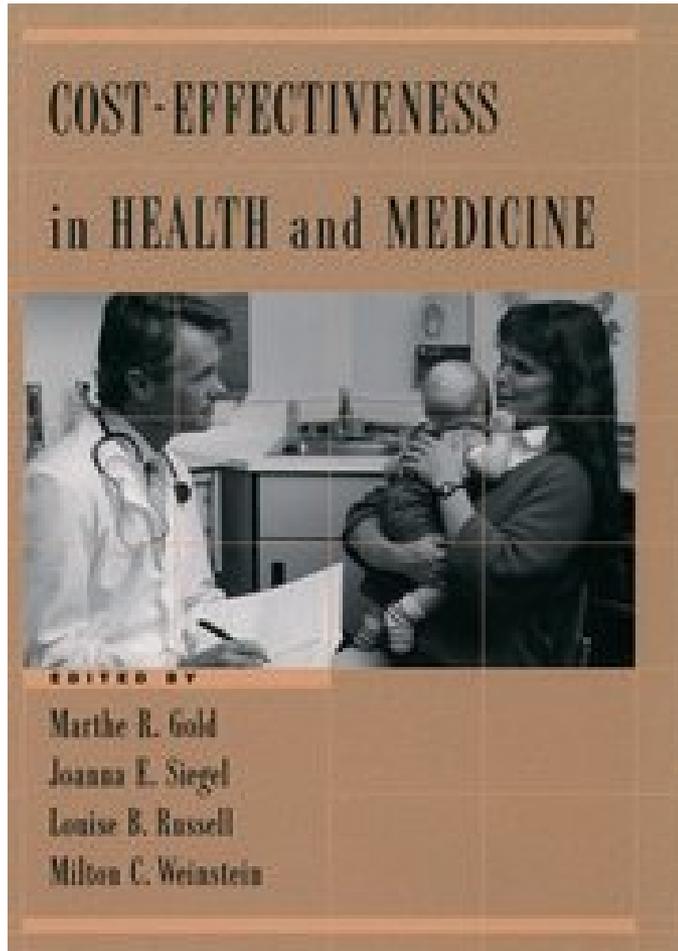


Interactive question

- Have you read a cost-effectiveness analysis?
 - Yes/no

- Have you helped conduct a cost effectiveness analysis?
 - Yes/no

Original Panel



- “The Gold Book” — 1996
- Recommendation for reference case
- Emphasis on cost/QALYs
- Became standard reference for CEA, cited more than 8,000 times

Goal of 2nd Panel on CEA

- Update recommendations for conduct of CEA
- Facilitate the CEAs that are:
 - Fair and transparent
 - Promote comparability
 - State-of-the art methods

2nd Panel

CO-CHAIRS:

Peter Neumann (Tufts Medical Center)

Gillian Sanders Schmidler (Duke)

Anirban Basu (U Washington)

Doug Owens (VA/Stanford)

Dan Brock (Harvard)

Lisa Prosser (U Michigan)

David Feeny (McMaster)

Josh Salomon (Harvard)

Murray Krahn (U Toronto)

Mark Sculpher (U York)

Karen Kuntz (U Minnesota)

Tom Trikalinos (Brown)

David Meltzer (U Chicago)

LEADERSHIP GROUP:

Peter Neumann, Gillian Sanders, Ted Ganiats (UC San Diego),
Joanna Siegel (AHRQ/PCORI), Louise Russell (Rutgers)





Overview of Key Recommendations

JAMA[®]

The Journal of the American Medical Association

JAMA | Special Communication

Recommendations for Conduct, Methodological Practices, and Reporting of Cost-effectiveness Analyses Second Panel on Cost-Effectiveness in Health and Medicine

Gillian D. Sanders, PhD; Peter J. Neumann, ScD; Anirban Basu, PhD; Dan W. Brock, PhD; David Feeny, PhD;
Murray Krahn, MD, MSc; Karen M. Kuntz, ScD; David O. Meltzer, MD, PhD; Douglas K. Owens, MD, MS;
Lisa A. Prosser, PhD; Joshua A. Salomon, PhD; Mark J. Sculpher, PhD; Thomas A. Trikalinos, MD;
Louise B. Russell, PhD; Joanna E. Siegel, ScD; Theodore G. Ganiats, MD

September 13, 2016

A COMPLETE UPDATE AND REVISION OF THE LANDMARK TEXT

COST- EFFECTIVENESS IN HEALTH AND MEDICINE

SECOND EDITION

EDITED BY

Peter J. Neumann, Gillian D. Sanders,
Louise B. Russell, Joanna E. Siegel,
and Theodore G. Ganiats

OXFORD

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- 1. Using CEA**
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12. Ethical considerations **(NEW)**
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Overview

- 1. Reference cases and impact inventory**
- 2. Costs**
- 3. Valuing health outcomes**
- 4. Modeling**
- 5. Uncertainty analysis**
- 6. Reporting**



The Reference Case and Impact Inventory

Recommendations of the
Second Panel on Cost-Effectiveness
in Health and Medicine



Original Panel's Recommendations

- Reference Case
- Societal Perspective
- Consider all parties affected
- Address specific decision contexts as needed

Experiences since the Original Panel

- Many CEAs, most not using the societal perspective
- Even when stating using societal perspective – important elements often omitted
- Decision makers using CEA – often have taken more focused perspective



Perspective: Second Panel's Considerations

- Appeal of societal perspective
- Is there a single “societal perspective”?
- Need to promote quality and comparability

Recommendation – Reference Cases:

- All studies represent a reference case analysis based on a **health sector perspective** and a reference case based on a **societal perspective**
- Measure health effects in QALYs
- Intended to enhance consistency and comparability

Recommendation: Health Sector Perspective

- Results should be summarized in ICER
- NMB and NHB may also be reported
- Range of CE thresholds should be considered

Recommendation: Impact Inventory

- Include impact inventory table which lists the health and non health impacts of an intervention
- Ensures that all consequences, including outside the formal healthcare sector, are considered
- Provides a framework for organizing, thinking about, and presenting various types of consequences

The Impact Inventory

Sector	Type of Impact (list category within each sector with unit of measure if relevant) ^a	Included in This Reference Case Analysis From...Perspective?		Notes on Sources of Evidence
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Sections of the Impact Inventory divide consequences across:

- **Formal healthcare sector**



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- **Informal healthcare sector**



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Sector	Type of Impact (list category within each sector with unit of measure if relevant) ^a	Included in This Reference Case Analysis From...Perspective?		Notes on Sources of Evidence
		Health Care Sector	Societal	
Formal Health Care Sector				
Health	Health outcomes (effects)			
	Longevity effects	<input type="checkbox"/>	<input type="checkbox"/>	
	Health-related quality-of-life effects	<input type="checkbox"/>	<input type="checkbox"/>	
	Other health effects (eg, adverse events and secondary transmissions of infections)	<input type="checkbox"/>	<input type="checkbox"/>	
	Medical costs			
	Paid for by third-party payers	<input type="checkbox"/>	<input type="checkbox"/>	
Paid for by patients out-of-pocket	<input type="checkbox"/>	<input type="checkbox"/>		
Future related medical costs (payers and patients)	<input type="checkbox"/>	<input type="checkbox"/>		
Future unrelated medical costs (payers and patients)	<input type="checkbox"/>	<input type="checkbox"/>		
Informal Health Care Sector				
Health	Patient-time costs			
	Unpaid caregiver-time costs			
	Transportation costs			
Non-Health Care Sectors (with examples of possible items)				
Productivity	Labor market earnings lost			
	Cost of unpaid lost productivity due to illness			
	Cost of uncompensated household production			
Consumption	Future consumption unrelated to health			
Social Services	Cost of social services as part of intervention			
Legal or Criminal Justice	Number of crimes related to intervention			
	Cost of crimes related to intervention			
Education	Impact of intervention on educational achievement of population			
Housing	Cost of intervention on home improvement (eg, removing lead paint)			
Environment	Production of toxic waste pollution by intervention	NA	<input type="checkbox"/>	
Other (specify)	Other impacts	NA	<input type="checkbox"/>	

Type of Impact (list category within each sector with unit of measure if relevant) ^a	Included in This Reference Case Analysis From...Perspective?	
	Health Care Sector	Societal
Patient-time costs	NA	<input checked="" type="checkbox"/>
Unpaid caregiver-time costs	NA	<input checked="" type="checkbox"/>
Transportation costs	NA	<input checked="" type="checkbox"/>



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	Health-related quality-of-life effects	<input type="checkbox"/>	<input type="checkbox"/>	
	Other health effects (eg, adverse events and secondary transmissions of infections)	<input type="checkbox"/>	<input type="checkbox"/>	
	Medical costs			
	Paid for by third-party payers	<input type="checkbox"/>	<input type="checkbox"/>	
Informal Health Care Sector	Paid for by patients out-of-pocket	<input type="checkbox"/>	<input type="checkbox"/>	
	Future related medical costs (payers and patients)			
	Future unrelated medical costs (payers and patients)			
	Non-Health Care Sectors (with examples of possible items)			
Productivity	Labor market earnings lost			
Productivity	Cost of unpaid lost productivity due to illness			
Productivity	Cost of uncompensated household production			
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Type of Impact (list category within each sector with unit of measure if relevant) ^a	Included in This Reference Case Analysis From...Perspective?	
	Health Care Sector	Societal
Labor market earnings lost	NA	<input checked="" type="checkbox"/>
Cost of unpaid lost productivity due to illness	NA	<input checked="" type="checkbox"/>
Cost of uncompensated household production ^b	NA	<input type="checkbox"/>
Future consumption unrelated to health	NA	<input type="checkbox"/>
Cost of social services as part of intervention	NA	<input checked="" type="checkbox"/>
Number of crimes related to intervention	NA	<input type="checkbox"/>
Cost of crimes related to intervention	NA	<input type="checkbox"/>



Interactive question

- For which analysis would the societal perspective be most important?
 - Cost effectiveness of treatment for opioid use disorder
 - Cost effectiveness of use of implantable defibrillators to prevent sudden cardiac death

Quantifying and Valuing Non-health Components in the Impact Inventory

Analysts should attempt to quantify and value nonhealth consequences in the Impact Inventory unless those consequences are likely to have a negligible effect on the result of the analysis.



Summary and Disaggregated Measures

- Analysts should present the items listed in the impact inventory in the form of disaggregated consequences across different sectors.
- Use 1 or more summary measures, such as an incremental cost effectiveness ratio, net monetary benefit, or net health benefit, that include some or all of the items listed in the impact inventory.
- Identify which items are included and how they are measured and valued.



Valuing Costs

Recommendations of the
Second Panel on Cost-Effectiveness
in Health and Medicine



Valuing Costs: 2nd Panel Reference Cases

- **Healthcare sector reference case**
 - medical costs (current and future, related and unrelated) borne by third-party payers and paid for out-of-pocket by patients (i.e., **health sector costs**)
- **Societal reference case** also includes
 - time costs of patients in seeking and receiving care,
 - time costs of informal (unpaid) caregivers,
 - transportation costs,
 - effects on future productivity and consumption, and
 - other costs and effects outside the healthcare sector.



Valuing Health Outcomes

Recommendations of the
Second Panel on Cost-Effectiveness
in Health and Medicine



Valuing Health Outcomes

- Health Consequences should be aggregated into a single measure using QALYs
- Use Community Preferences
- For the Reference Case Recommend the Use of Generic Preference-Based Measures
- Panel did **not** recommend the use of one particular measure

Acknowledge the Potential Limitations of Generic Preference-Based Measures

In situations in which analysts have **empirical evidence** that relying on generic preference-based measures is less than ideal, or that the direct elicitation of scores for relevant health states from the general population is less than ideal, the analyst should incorporate alternative approaches.

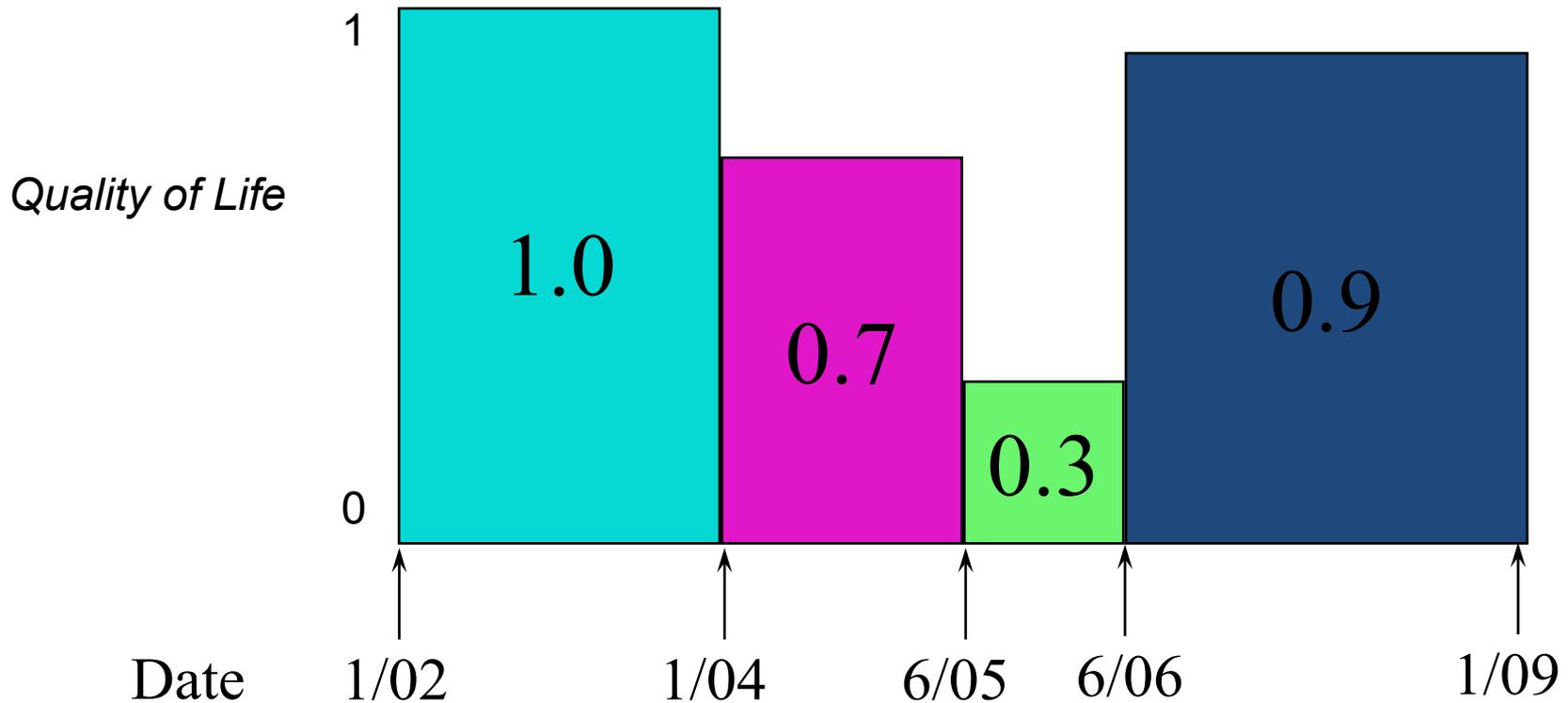
Situations in which this may arise include cases/contexts:

1. In which generic preference-based measures are known to lack responsiveness and/or cross-sectional construct validity;
2. There are important spillovers from the intervention such as effects on the health of caregivers and other members of the family;
3. It is difficult for those who have not experienced or observed the health states associated with the condition and/or its treatment to understand them sufficiently well to provide meaningful scores for those health states.

We therefore also recommend that community-derived preference weights be supplemented by preference scores elicited from patients when there are important concerns about the extent to which instruments based on community preferences can represent an informed social judgment about the desirability of a particular condition or outcome.



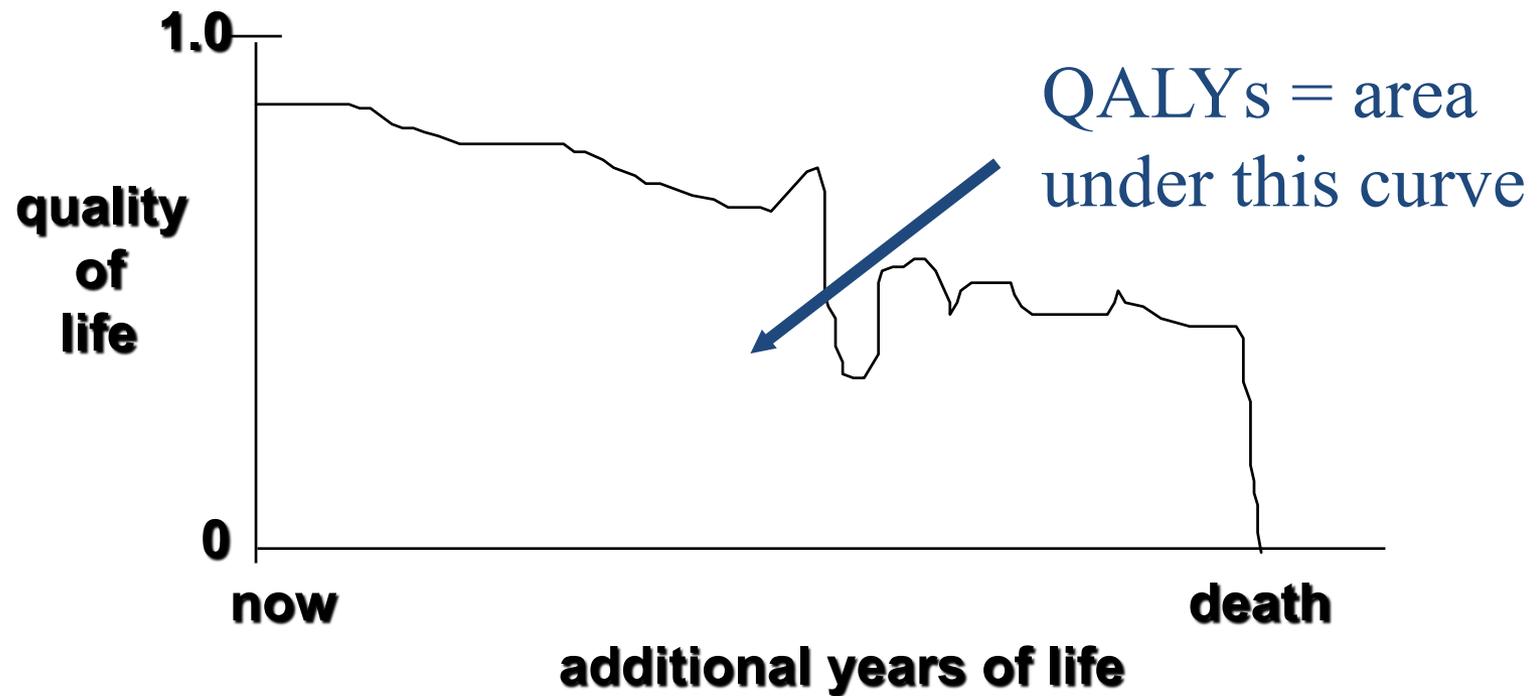
Calculating QALYS



Life Years = 2 + 1.5 + 1 + 2.5 = 7 years

QALYs = (2)(1)+(1.5)(.7)+(1)(.3)+(2.5)(.9) = 5.6 years

A “Life Path” of health-related quality of life



Consider the effect of intervention...



Conducting and Implementing CEAs

Recommendations of the
Second Panel on Cost-Effectiveness
in Health and Medicine



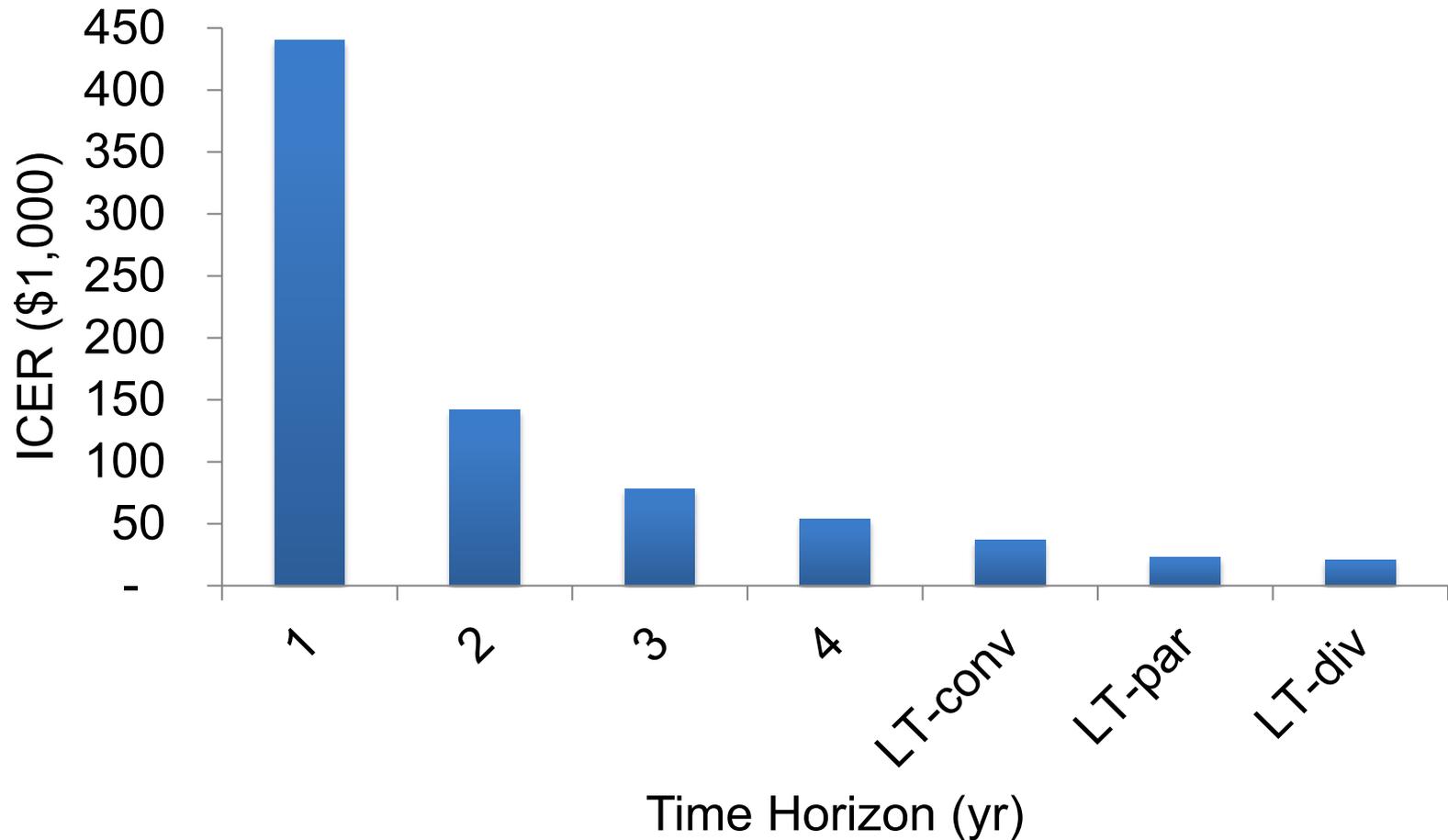
Protocol

- We recommend use of a written, publicly available **protocol** that specifies
 - Objectives, type of analysis
 - Perspective
 - Interventions and comparators
 - Population
 - Time horizon
 - Sources of data, key assumptions, analysis plan

Decision Model Usually Needed for Extrapolation

- Beyond the time horizon of available data
- From intermediate (surrogate) outcomes to long-term outcomes
- To population subgroups not observed in studies
- Long-term outcomes associated with diagnostic test strategies
- To strategies that have not been studied in head-to-head comparisons

ICERs Vary by Time Horizon



Hlatky et al. Clinical Trials 2006;3:543-51.



Key Modeling Recommendations

- Initial conceptualization of model should be independent of data identification phase
- Documentation and justification of structural assumptions should be provided
- Validation of model should occur throughout the conduct of a CEA

Uncertainty Analysis

- Propagation of input uncertainty informs on decision uncertainty
- Correlations among parameters should be considered
- Structural uncertainties should be explored (in scenario analyses if necessary)

Structural Uncertainty

- How to model the effects of an intervention beyond the time horizon of the data
- How different states of health and pathways of care are characterized in a model
- How disease progression is modeled over time (extrapolated) beyond the follow-up period of study
- Judgments about the relevance and appropriateness of different sources of evidence

Sensitivity Analysis

- Examining model outputs while conditioning on specific inputs provides insight about model behavior
 - One-way and multi-way sensitivity analyses
 - Threshold analyses
- Can be used as a means of understanding the implication of heterogeneity

Reporting CEAs

Recommendations of the
Second Panel on Cost-Effectiveness
in Health and Medicine



Reporting: Updated Recommendations

- Purpose
 - Transparency
 - Completeness
 - Comparability
- Key Updates
 - Structured abstract
 - Impact inventory
 - Intermediate outcomes
 - Disaggregated results

Structured Abstract Format

- Objective
- Intervention
- Target Population
- Perspectives
- Time horizon
- Discount rate
- Costing year
- Study Design
- Data sources
- Outcome Measures
- Results of base-case analysis
- Results of uncertainty analysis
- Limitations
- Conclusions

Elements to include in Standard Abstract Format

- Objective
- Methods
 - ✓ Intervention
 - ✓ Target Population
 - ✓ Perspectives
 - ✓ Time horizon
 - ✓ Discount rate
 - ✓ Costing year
 - ✓ Study Design
 - ✓ Data sources
 - ✓ Outcome Measures
- Results
 - ✓ Results of base-case analysis
 - ✓ Results of uncertainty analysis
 - ✓ [Limitations]
- Conclusions



Reporting Checklist

Introduction

- Background of the problem

Study Design and Scope

- Objectives
- Audience
- Type of Analysis
- Target population(s)
- Description of interventions & comparators
- Boundaries of the analysis (scope)
- Time horizon
- Analytic perspectives
- Whether this analysis meets the requirements of the reference case
- Analysis plan

Methods & Data

- Trial-based analysis or model based (plus additional descriptors)
- Key outcomes
- Complete information on data sources
- Methods for obtaining estimates of effectiveness /evidence synthesis
- Methods for estimating costs & preference weights
- Critique of data quality
- Costing year
- Method used to adjust costs
- Type of currency
- Source and methods for obtaining expert judgment
- Discount rate(s)



Reporting Checklist, cont.

Impact Inventory

- Full accounting of consequences within and outside of the health sector

Results

- Results of model validation
- Reference case results: total costs & effectiveness, incremental costs & effectiveness, ICERs, measure(s) of uncertainty
- Disaggregated results for important categories of costs and/or outcomes
- Sensitivity analysis, other estimates of uncertainty
- Graphical representation of cost-effectiveness results & uncertainty analysis
- Aggregate cost and effectiveness information
- Secondary analyses

Disclosures

- Statement of any potential conflicts of interest relating to funding source, collaborations, or outside interests

Discussion

- Summary of reference case results
- Summary of sensitivity of results to assumptions and uncertainties in the analysis
- Discussion of the study results in the context of related CEAs
- Discussion of ethical implications
- Distributive implications of an intervention
- Limitations of the study
- Relevance of study results to specific policy questions or decisions

Reporting: Summary

- Continued emphasis on transparency: enough detail should be provided to allow for replication
 - Structured abstract
 - Reporting checklist
 - Impact inventory
 - Intermediate outcomes & disaggregated results
 - Technical appendix
- New guidance on conflict of interest
- Going forward: sharing models/data, new formats for presenting results, communicating results in an era of emerging technologies

Valuing Health Outcomes

- Health Consequences should be aggregated into a single measure using QALYs
- Use Community Preferences
- For the Reference Case Recommend the Use of Generic Preference-Based Measures
- Panel did **not** recommend the use of one particular measure

Second Panel Recommendations: Summary of Key Changes

- Two reference cases instead of one:
 - Healthcare sector
 - Societal
- Use of impact inventory to show outcomes included/excluded
- New recommendations on modeling, ethical considerations, reporting

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Recommendations for Conduct, Methodological Practices, and Reporting of Cost-effectiveness Analyses Second Panel on Cost-Effectiveness in Health and Medicine

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A COMPLETE UPDATE AND REVISION OF THE LANDMARK TEXT

COST- EFFECTIVENESS IN HEALTH AND MEDICINE

SECOND EDITION

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