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

Kevin Frick, PhD

Johns Hopkins University
Section A

What a Reader of a CEA Should See
History of the Panel

Established to support the conduct and interpretation of cost-effectiveness studies in prevention

★ *Eventually expanded to CEA in general*

Improve and standardize methodology

Set of guidelines were called a “reference case”

While guidelines help, the field continues to develop and debates continue to occur
Framework

Background of the problem
General framing and design of the analysis
Target population for the intervention
Other program descriptors
Description of comparator programs
Boundaries of the analysis
Statement of the perspective of the analysis
Data and Methods

Description of the “event pathway”

Identification of outcomes of interest

Description of the model used

Modeling assumptions
Data and Methods

Description of the “event pathway”

Identification of outcomes of interest

Description of the model used

Modeling assumptions
Diagram of “event pathway”/model

Software used

Complete information of sources of effectiveness data, cost data, preference weights
Methods for obtaining estimates of effectiveness, cost, and preferences

Critique of data quality

Statement of year of costs

Statement of method used to adjust costs for inflation

Continued
More on Data and Methods

Statement of type of currency

Source and methods for obtaining expert judgment

Statement of discount rates
Results of model validation

Reference case results

- Discounted and undiscounted
- Total costs and effectiveness
- Incremental costs and effectiveness
- Incremental cost-effectiveness ratios

Results of sensitivity analyses

Continued
Results of model validation

Reference case results

- Discounted and undiscounted
- Total costs and effectiveness
- Incremental costs and effectiveness
- Incremental cost-effectiveness ratios

Results of sensitivity analyses
More about Results

Other estimates of uncertainty, if available

Graphical representation of results

Aggregate cost and effectiveness information

Disaggregated results, as relevant

Secondary analyses using 0% and 5% discount rates

Other secondary analyses, as relevant
Summary of reference case results

Summary of sensitivity of results to assumptions and uncertainties in the analysis

Discussion of analytic assumptions with important ethical implications

Limitations of the study

Relevance of study results for specific policy questions or decisions
More Discussion Points

Results of related cost-effectiveness analyses
- *Reporting on the previous literature*

Distributive implications of an intervention
- *Are there winners and losers?*
Section B

Panel on Cost-Effectiveness Recommendations
The list of alternative strategies should include all reasonable options and a baseline comparison.

Continued
The definition of all reasonable is a non-negligible issue

- Should it be anything that can be dreamed up?
- Should it be alternatives that are politically feasible?
- Should it be limited to alternatives within a fixed budget?
Report Societal Perspective

Others may be the primary reason for undertaking a study

- *Show the analysis from the perspective that is the primary function*
Also Show the Societal Perspective

Having a portfolio of societal perspective studies will make it easier to compare across disease areas or treatments
What to Include in a Societal Perspective?

All measurable opportunity costs, representing all groups affected by a program, should be included in the societal perspective

- Opportunity cost is often approximated by the market price
- The technical definition is the value of the next best alternative use of the resources
Capture the length of the program and the time during which all costs, harms, and benefits occur

- A program preventing under five mortality should be evaluated considering the five years of implementation during which mortality is to be prevented and the remainder of the lifetime of the children who do not die as a result of the program.
Costs can be referred to as average, incremental, or marginal.

An average cost-effectiveness analysis would compare all alternatives to doing nothing or the least expensive to get an average cost per better outcome gained.
How to Characterize Costs and Effects?

Incremental analysis focuses on the cost per outcome for an alternative in comparison to the next most expensive and effective.

Marginal analysis focuses on increasing the size of a program a small amount.
How Comprehensive Should the Analysis Be?

Include all benefits and harms that have a meaningful impact on the results

- Costs and effects may vary greatly or vary slightly
How Comprehensive Should the Analysis Be?

Include all benefits and harms that have a meaningful impact on the results

- *Costs and effects may be large or small in magnitude*
  - Highly variable and large in magnitude should be included
  - Little variation and small in magnitude can be excluded
  - Others must be considered individually
Sensitivity Analyses

Univariate

Change the value of one variable used in the analysis and see what happens to the results
Multivariate

Change the value of more than one variable at a time and see what happens to the results

- Many variations on how to change the values of multiple variables in order to get some idea of how stable the results are
Implications of the Results

Distributional

- Who has higher costs?
- Who saves money?
- Who has better outcomes?
- Does anyone have worse outcomes?
Implications of the Results

Ethical

★ Are the effects distributed in a way that is fair?
★ How do the results match with standard ideas of values and ethics?
What Should be Explicit?

All assumptions

All sources of information
Incidence-Based Costs

Costs of a disease starting from the onset and continuing for the remainder of the person’s life

Not all the costs are associated with a condition in a given year
Cost Methods

Resource value rather than charges if they are different

Human capital approach to estimate productivity costs
Health Utility Values

Utility assessment should be preference-based and interval scaled on a scale with optimal health equal to one and death equal to zero.

Generic health instrument

Community-based scoring algorithm
Discounting

Discount future costs and health

Discount both future costs and health at same rate

Use discount rate of 3% with sensitivity analyses over a reasonable range, especially 0% and 5%
For CBA, capture all costs and benefits and report a net benefit

Use final health outcome measures in a CEA

The numerator should include only direct costs unless the quality of life instrument in the denominator does not include indirect costs
QALY measure should rely on community preferences and preference based weights that use an interval scale.
The numerator of a CEA should include costs of the intervention, adverse health outcomes, and health outcomes averted by the intervention

- *Medical, non-medical, possibly productivity*

Report total costs, total effect, incremental costs, incremental effects, and incremental cost-effectiveness ratios
Section C

Panel on Cost-Effectiveness Recommendations in Action
Healthy Start: Background

Program set up in most socioeconomically disadvantaged areas of the city

Neighborhood canvassing for expectant mothers

Case management during pregnancy

Follow-up for mother, father, and children after pregnancy until children turn three
Healthy Start: Problems Being Addressed

Avoiding very low birth weight

Help mother to get her life in order

- Complete education
- Get a job
- Avoid interaction with criminal justice system
- Better parenting (less abuse and neglect)
- Become economically independent
Help fathers

- Similar list of issues as for mothers
- Emphasize playing a role in children’s lives
**Healthy Start Data**

Very low birth weight

- *Easily tracked*

Effects of very low birth weight

- *No link to Medicaid*
- *No plan (when program began) to link up with data for child abuse or early education programs*
Follow-up data on mothers and fathers other than simple administrative data was quite limited.
## Healthy Start Time Horizon

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<th>Data Availability</th>
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### Healthy Start Perspective

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Healthy Start Summary

Most readily available data and literature sources to look at one year costs related to very low birth weight avoided from a governmental perspective

Very low birth weight occurs in only 4% of the population at risk even without an intervention
Significant reduction in very low birth weight

However, even with an expensive condition like VLBW, the costs exceeded the benefits
Healthy Start Sensitivity Analyses

Consider longer time periods

Think about other potential benefits

Treat as a cost-effectiveness analysis where the governmental calculations really represent only the numerator of the cost-effectiveness analysis