IOM Recommendations for Valuing Health in Regulatory Cost Effectiveness Analysis

NIH Workshop
Economic Analysis of Nutrition Interventions: Methods, Research and Policy

February 23, 2010
Bethesda, MD

Wilhelmine Miller, MS, PhD
George Washington University
Department of Health Policy

Overview

- Context
  - OMB directive for CEA in regulatory analysis
  - Precedents in health care and public health

- Charge to the Study Committee

- Committee's Criteria and Conclusions

- Recommendations
  - Choosing an integrated measure
  - Valuing and calibrating health states
  - Constructing and reporting CE ratios
  - Use of QALYs in BCA
  - Information for regulatory decisions
  - Public participation
  - Data collection and research

- Current Federal Agency Practices

- Considerations Moving Forward

VALUING HEALTH
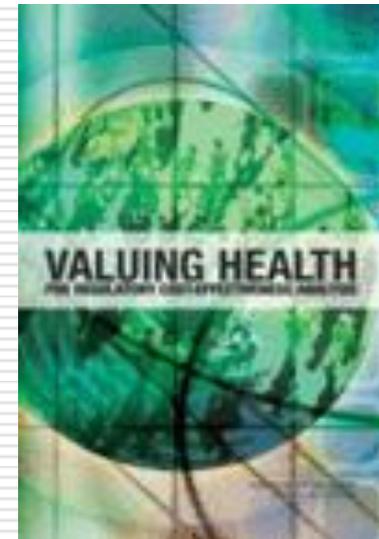
Context
OMB Directive for CEA in Regulatory Analysis

- ☐ Historically, benefit-cost analysis (BCA) has been the predominant approach used to assess the economic impacts of major U.S. health and safety regulations.

- ☐ In 2003 OMB issued *Circular A-4: Regulatory Analysis*, requiring that agencies *also* conduct cost-effectiveness analysis (CEA) whenever "a valid effectiveness measure can be developed."

- ☐ In 2004, OMB and several Federal agencies asked the Institute of Medicine to convene a consensus committee to consider technical and ethical issues related to the selection of integrated effectiveness measures.

- ☐ The IOM Committee to Evaluate Measures of Health Benefits for Environmental, Health, and Safety Regulation published its report in January 2006.

Context
# Precedents in Health Care and Public Health

- U.S. Panel on Cost-effectiveness in Health and Medicine was convened in 1993 by the Public Health Service to assess "state of the science" and to define best practices for conduct of cost-effectiveness analyses in health care

- *Cost-Effectiveness in Health and Medicine* (1996) has been influential; its recommendations for conducting and reporting CEAs have been adopted by many journals and practitioners as the standard approach

- Proposed that the "Reference Case Analysis" be conducted from the societal point of view

- Recommended that health-related quality-of-life (HRQL) weights used to estimate Quality-Adjusted Life Years (QALYs):
  - Reflect the general population's valuation of particular health states, not the values assigned only by people with the condition)
  - Be directly elicited through a time-trade-off or standard gamble exercise, or use pre-developed instrument or "index" with certain properties
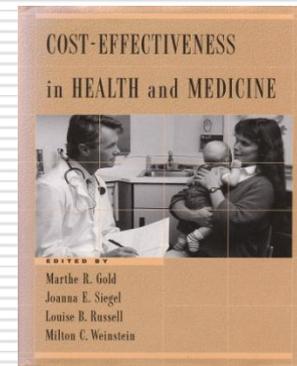
Committee Charge

- ☐ Describe current Federal agency practices for evaluating the costs and benefits of economically significant health and safety regulations

- ☐ Review benefit measures currently used in CEA that aggregate morbidity and mortality impacts (e.g., QALYs)

- ☐ Develop criteria for selecting measures for use in regulatory CEA

- ☐ Evaluate measures in terms of data needs, feasibility, validity, appropriateness for special populations, and ethical implications

- ☐ Recommend measures for use in regulatory CEA

- ☐ Conduct case studies that apply alternative measures using data from completed agency regulatory analyses

- ☐ Discuss criteria for identifying regulations for which CEA would be informative

- ☐ Recommend research to improve measurement of health benefits

In short…
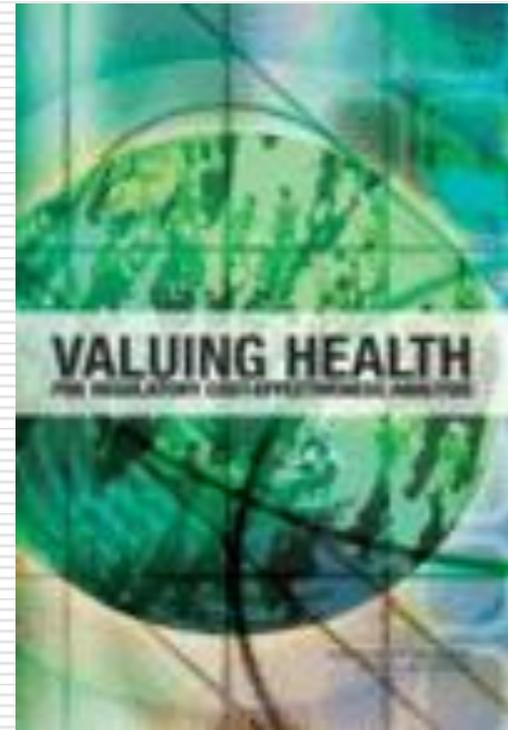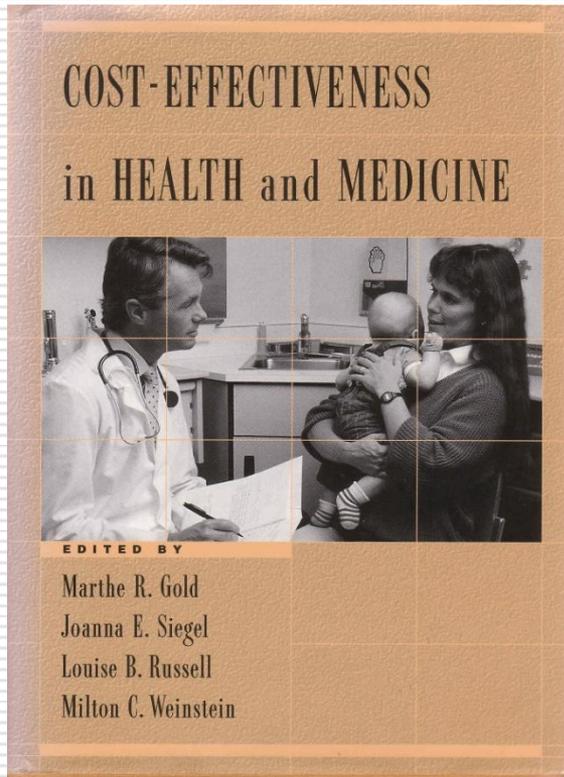
Criteria for Selecting Measures

- ☐ Measure should be applicable to the range of health states and conditions considered in regulatory analysis

- ☐ It should be sensitive to change, and not exhibit floor or ceiling effects

- ☐ Values should be derived from a sample of adequate size and be representative of the population affected by the costs and benefits of the regulation

- ☐ Measure should be acceptable to users and to the public, including those involved or interested in the regulatory development process

- ☐ It should be practical for use in regulatory analysis and as inexpensive to use as is compatible with other objectives

Committee Conclusions

- CEA, like BCA, is a useful tool for developing and assessing regulatory interventions to improve health and safety

- The information from CEA (or BCA) alone is not sufficient to inform regulatory decisions; other types of research and public involvement are also necessary

- Although regulatory CEA is feasible today, additional data and methodological improvements would improve its quality and usefulness

- Greater consistency in analytic practices and reporting across agencies would increase the transparency and comparability of the results and lead to better informed decisions

- Comparisons of CE ratios for diverse interventions can be misleading if they do not highlight differences in methods, unmeasured effects, and distributional impacts

Recommendations
Choosing an Integrated Measure

- ☐ Among the possible health-adjusted life year measures, the QALY is most appropriate in regulatory analysis
  - ■ It is simple, in wide use, and the most extensively evaluated HALY metric
  - ■ If not based on direct preference elicitations for the health states of interest, QALY estimates should be based on generic health-related quality of life indexes, such as the EuroQol EQ-5D, the Health Utilities Index, the Quality of Well-being Scale, or the SF-6D

- ☐ Preference-based EQ-5D index values have been estimated for the US population recently, making this the "leading candidate" index *for the time being*

Valuing and Calibrating Health States

- ☐ Life-year and QALY estimates should reflect actual population health as closely as possible, comparing the estimated HRQL and life expectancy of the affected population in the regulatory baseline to the predicted, post-intervention estimates

  - ■ Descriptive information on quality of life and longevity impacts should be derived from those who have experienced the effects; i.e., patients rather than medical experts

  - ■ Values (weights) for different health states should be derived from the population affected by the costs, benefits, or other impacts of the regulatory intervention, often best represented by the general U.S. population

  - ■ Predicted health status in the absence of the condition of concern should reflect expected actual health, not perfect or optimal health

Constructing and Reporting CE Ratios

- ☐ Report multiple cost-effectiveness ratios
  - ■ Compliance cost per death averted
  - ■ Compliance cost per life year gained
  - ■ Health-benefits-only ratio, using QALYs
  - ■ Comprehensive ratio using QALYs, with other benefits incorporated as offsets to costs
- ☐ Incremental CE ratios are generally the most useful summary measure for comparing different regulatory interventions

- ☐ In addition to reporting effects in the aggregate, QALY estimates should be reported separately for each health impact. Cases of disease avoided and cause-specific mortality should also be reported

- ☐ Information on related uncertainties and on non-quantified effects should accompany all reported CEA results

Recommendations
Use of QALYs in BCA

- ☐ Regulatory analyses should not assign monetary values to QALY estimates as a method for valuing health states

  - ■ While monetized QALYs may be necessary for BCAs because WTP values are lacking in the short term, this practice should be discouraged

  - ■ Neither theoretical justification nor a consensus exists for establishing a $-per-QALY value

- ☐ Dollar valuation for a QALY lacks theoretical or empirical support

  - ■ QALYs are usually monetized based on a constant value, on convention or are derived from estimates of the value of statistical life year (VSLY)

  - ■ Recent reviews reject the notion of a constant VSLY

  - ■ Economic theory and the limited empirical work available suggest that the value of QALY will vary depending on the characteristics of the affected population and of the risk itself

Recommendations
Information for Regulatory Decisions

- ☐ The process for making regulatory decisions should explicitly address and reflect distributional, ethical, and other non-quantified implications of a proposed intervention

- ☐ For example, do the risks have attributes that affect their value but are not reflected in the quantified valuation measures?

  - ■ Risks not subject to personal control
  - ■ Risks especially dreaded
  - ■ Risks undetectable by the senses
  - ■ Risks have delayed effects
  - ■ Risks not well understood

Recommendations
Information for Regulatory Decisions

☐ Do pre-regulatory or post-regulatory costs or risks disproportionately affect certain population groups?

- ■ Future generations
- ■ Infants, young children
- ■ Elderly people
- ■ Persons with disabilities or preexisting conditions
- ■ Especially vulnerable groups to the risk
- ■ Members of minority groups
- ■ People with low incomes
- ■ Geographically concentrated groups

☐ The subgroups selected for comparative analysis of impacts anticipate what is relevant for justice; it presumes some kind of disproportionality in benefits and/or burdens

Recommendations
Public Involvement

- [ ] Policy makers and agency administrators should involve a broad range of individuals and groups at all stages of policy development for regulating risks. Mechanisms include:
    - Notice and comment
    - Public hearings
    - Participatory workshops
    - Advisory committees
    - Citizen juries

- [ ] Greater consistency and transparency in presenting analytic results will facilitate—but not guarantee—public understanding and participation

- [ ] Benefits and limitations of public engagement in regulatory context:
    - Time consuming/expensive
    - Raises expectations without delivering
    - Subject to capture by powerful interest groups
    - Gives voice and recognition to underrepresented groups
    - Raises awareness of need/potential for remediation
    - Surface new solutions because of greater diversity and local knowledge

Recommendations
Data Collection and Research

- HHS and regulatory agencies with health-related portfolios should buttress data collection efforts to improve the quality of the effectiveness information available for conducting regulatory CEAs

- Better coordination of research priorities across federal agencies and within HHS to support CEA is needed

- Research emphasis should be placed on building additional methods for capturing and valuing HRQL, and on increasing the understanding and versatility of existing measures

Recommendations
Data Collection and Research

- ☐ Improvements in data used to assess health risks addressed by regulatory interventions should be a research priority
  - ■ Committee's case studies demonstrated the importance of an adequate epidemiologic base to estimating the health impacts (morbidity and mortality) of abating or mitigating risk through regulation

- ☐ HHS and other agencies should collect HRQL information through routinely administered population health surveys and other major risk assessment and monitoring data collection efforts

Recommendations
Data Collection and Research

- ☐ HHS should, with other federal agencies, coordinate the development of an integrated research agenda to improve the quality, applicability, and breadth of integrated measures for use in regulatory CEA

- ☐ Priorities include:
    - ■ Improving methods for eliciting societal values for investments in health
    - ■ Developing methods for measuring and valuing children's HRQL
    - ■ Building methods to correlate QALY values based on different generic indexes so that estimates from different existing studies can be combined in the same analysis

Current Federal Agency Practices

- The U.S. Environmental Protection Agency promulgates the majority of the economically significant health and safety regulations
  - EPA relies primarily on BCA and has provided only "illustrative" CEAs in response to OMB requirements

- The Food and Drug Administration and National Highway Traffic Safety Administration also have on-going regulatory programs affected by these recommendations, other agencies promulgate regulations less frequently
  - FDA and NHTSA have historically reported CEA as well as BCA results

- EPA does not use monetized QALYs in its BCAs, but they are commonly used in FDA and NHTSA analyses
  - WTP estimates are lacking for most nonfatal health effects of concern

Current Federal Agency Practices

- ☐ Agency staff agreed with most recommendations but implementation inhibited by time, resource constraints and competing priorities

- ☐ OMB has not issued guidance on implementation of IOM recommendations

- ☐ Most controversial is the IOM Committee's recommendation *not* to use of monetized QALYs in BCA

- ☐ Some find that QALY estimates and CE ratios are more easily understood and widely accepted by decision makers than are willingness-to-pay measures and BCA results

- ☐ Others argue that WTP/BCA is the correct conceptual framework

- ☐ Recommendation to report multiple measures is also problematic (When is more and more disaggregated information too much information?)

Considerations Moving Forward

- ☐ Assess informational needs of decision makers

- ☐ Develop criteria for matching particular measures to the circumstances in which those measures are most useful, instead of routinely reporting numerous results

- ☐ Promote cross-agency collaboration:
  - ■ Develop formal partnerships or informal agreements to fund different components of key projects
  - ■ Create forums for sharing information and discussion
  - ■ Consider applications in health care services research along with regulatory applications

- ☐ Separate funding for improved data and methods from specific rulemakings

IOM Committee to Evaluate Measures of Health Benefits for Environmental, Health, and Safety Regulation

- ☐ Robert Lawrence, MD, Chair
- ☐ Henry Anderson, MD
- ☐ Richard Burnett, PhD
- ☐ Carl Cranor, PhD
- ☐ Maureen Cropper, PhD
- ☐ Norman Daniels, PhD
- ☐ Dennis Fryback, PhD
- ☐ Alan Garber, MD, PhD
- ☐ Marthe Gold, MD, MPH
- ☐ James Hammitt, PhD
- ☐ Lisa Iezzoni, MD, MSc
- ☐ Peter Jacobson, JD, MPH

- ☐ Emmett Keeler, PhD
- ☐ Willard Manning, PhD
- ☐ David Schkade, MBA, PhD

Study Director
    Wilhelmine Miller, MS, PhD
Principal Consultant
    Lisa Robinson, MPP
Advisers
    Judith Wagner, PhD
    Alan Krupnick, PhD
    Milton Weinstein, PhD

Case studies available at www.iom.edu