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Panel Issues Standards For Cost-Effectiveness Analysis

"The Reference Case consists of a standard set of methodological practices and assumptions which, if adopted, would produce a level playing field on which programs can compete for claims on scarce health-care resources."

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Physicians, health-care managers, consumers, business leaders, and government officials often speak of the need to make health care more "cost-effective," but there has been far from a consensus on exactly what this means. An important step toward consensus took place in June of this year, when the Panel on Cost-Effectiveness in Health and Medicine reported its findings to the federal government on the practice of cost-effectiveness analysis. This issue of **RISK IN PERSPECTIVE** discusses the work of the Panel.

Created by the Office of Disease Prevention and Health Promotion in the U.S. Public Health Service, the Panel was charged with developing recommendations on the roles, conduct, and reporting of cost-effectiveness analyses and with developing a set of standard analytical practices that would make cost-effectiveness analyses more comparable with one another. Milton C. Weinstein, Henry J. Kaiser Professor of Health Policy and Management, Harvard School of Public Health (HSPH) and Director of the Harvard Program on Economic Evaluation of Medical Technology (PEEMT), co-chaired the Panel. Joanna E. Siegel, Assistant Professor of Maternal and Child Health at HSPH, co-directed the project for the government during a leave of absence from Harvard. Professor Louise B. Russell of Rutgers University was the other co-chair, and Dr. Marthe R. Gold was the project director. The Panel consisted of economists, physicians, decision scientists, psychologists, epidemiologists, and ethicists with expertise and experience in cost-effectiveness analysis.

Several of the Panel's recommendations concerned the roles and limitations of cost-effectiveness analysis. Significantly, the Panel's report emphasizes that cost-effectiveness analysis is an aid to decision making, not a self-contained algorithm for allocating resources. Thus, the Panel tried to allay fears that a master computer in Washington would be programmed to determine health-care benefits based on mechanistically calculated cost-effectiveness ratios.

Much of the Panel's work revolved around the need to make cost-effectiveness analyses more comparable to one another. The "bottom line" in a cost-effectiveness analysis is a ratio of cost to health effect (e.g., $50,000 per quality-adjusted life year saved) which has meaning only by comparison to cost-effectiveness ratios for programs that compete for the same resources. Many analysts report cost-effectiveness ratios in so-called "league tables," in which programs or health interventions are ranked in priority order from the lowest to the highest ratios. Problems arise if different studies adopt different perspectives (e.g., societal, employer, or insurer), include different costs or effects in the numerator or denominator of the ratio, use different outcome measures or time discount rates, adopt different procedures for considering the effects of illness on productivity, treat uncertainty differently, or use different comparison programs. Brown and Fintor (1993), in a review of published cost-effectiveness analyses of screening mammography for breast cancer, found that what appeared to be a thirty-fold variation in the estimated cost per life year gained ($3,000 to $80,000) actually reduced to less than a factor of two after standardizing their methodological assumptions.

The Cost-Effectiveness Panel encourages more uniform analytic practices by defining a "Reference Case" which the Panel urges all analysts to include in cost-effectiveness studies. The Reference Case consists of a standard set of methodological practices and assumptions which, if adopted, would produce a level playing field on which programs can compete for claims on scarce health-care resources. In developing its methodological recommendations, the Panel appealed to theoretical principles from welfare economics and decision theory, practical considerations such as data availability, and ethical considerations. When theory and other considerations led to divergent or ambiguous conclusions, a Reference Case assumption was chosen nonetheless for the sake of convention.

The Reference Case analysis always adopts the societal perspective, which includes all benefits, harms, and costs regardless of who bears them. This does not preclude cost-effectiveness analyses that adopt other perspectives, such as that of a managed care organization, a government agency, or a hospital, but the

recommendation is that analyses intended also to inform broader resource allocations should report results for the Reference Case as well.

Most of the Panel's recommendations reaffirm what many analysts have long regarded as good practice. For example:

- The time horizon of an analysis should be long enough to incorporate relevant future effects of an intervention, even if modeling is required to extend outcomes past the time frame in a clinical trial or other source of primary data.
- Cost-effectiveness analyses are always incremental. The intervention of interest should be compared not only to existing practice but also to other potentially cost-effective options such as doing nothing, a viable low-cost option, and a best-available alternative. Failure to do so may lead to an unwarranted conclusion that an intervention is cost-effective, but only by comparison with a cost-ineffective comparator.
- The measure of health effectiveness in the Reference Case is quality-adjusted life years (QALYs). The valuation of health-related quality of life should be based on preferences, not summated scales such as the SF-36.
- Costs should be based on opportunity cost of resources, but reasonable approximations may be derived from cost-accounting systems or from prices with suitable adjustment for ratios of cost to charge. All costs should be expressed in constant dollars. Uncompensated costs such as the time spent receiving an intervention by patients or their families should be included among the resource costs of care.
- All costs and health effects should be discounted to present value. Based on recent economic indicators of long-term returns on investment, the Panel recommends a real discount rate of 3% for the Reference Case. To ensure comparability with existing analyses, a discount rate of 5% (which has been used in more than 80% of published CEAs, according to a recent review by PEEMT's Peter Neumann) should also be used.

Other Panel recommendations represent attempts to reconcile significantly more divergent practices to date. For example:

- Life years added by an intervention should always be adjusted for health-related quality of life. The implication is that analyses of cost per life year, even for interventions that have no effect on quality of life, are *not* Reference Case analyses. Data on age-specific health-related quality of life in the target population should be used to adjust for quality of life in added years.
- Preference weights for QALYs should be interval-scaled, meaning that the ratios of differences between values are meaningful. Despite controversy as to whether rating scales are suitable as interval-scaled preferences, the Panel accepted weights based on standard gambles, time tradeoffs, person tradeoffs, magnitude estimation, and rating scales for the Reference Case.
- Health-related quality of life should be based on established health-state classification schemes, not ad hoc sets of states. Examples of recommended systems include the McMaster Health Utilities Index, the Rosser Disability-Distress Index, the EuroQOL, and the Quality of Well-Being Scale. This recommendation was based on the need for uniformity and reproducibility.
- Preference weights should be obtained from the general community, not patients or providers. The rationale is that decisions involving resource allocation should be based on preferences from the perspective of persons who do not know what illnesses may affect them. All of the above scales incorporate community weights.
- So-called "indirect costs and benefits" associated with productivity should not be included in the numerator of a Reference Case cost-effectiveness ratio. Effects on personal income are already reflected in the quality-of-life weights used in QALYs. Short-term costs borne by employers and others (e.g., "friction costs") can be included in the numerator, and should be if they are important. Likewise, the costs of patients' time receiving health-care interventions should be included.
- The inclusion of future costs of unrelated diseases is left to the discretion of the analyst. More theoretical research is needed to resolve this issue, despite important recent papers on the topic. (The controversy is whether it is necessary to include *non-health* consumption costs along with health-care costs in order to be theoretically consistent.) In the meantime, Reference Case analyses may either include or exclude these costs, but sensitivity analyses should be performed to indicate whether their exclusion would materially affect the cost-effectiveness ratio(s).

All of the Panel's recommendations are meant to be subject to a "rule of reason." In other words, if satisfying one of the recommendations would require more effort or cost than would be justified by the increased precision and accuracy of the analysis, the analyst has discretion to compromise.

The full report of the Panel on Cost-Effectiveness in Health and Medicine has been published in a book, *Cost-Effectiveness in Health and Medicine*. A summary of the report will be contained in a three-part series in the *Journal of the American Medical Association* later this fall. A conference focusing on the Panel's report will be held in Bethesda, Maryland on November 25-26, 1996. For further information, call PHS Cost-Effectiveness Workshop, 301-650-0282.

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REFERENCE:

Brown ML, Fintor L: Cost-effectiveness of breast cancer screening: Preliminary results of a systematic review of the literature. *Breast Cancer Research and Treatment* 1993; 25:113–118.

FURTHER READING:

M.R. Gold, J.E. Siegel, L.B. Russell, M.C. Weinstein (eds.) *Cost-Effectiveness in Health and Medicine*. New York: Oxford University Press, 1996.

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COMING UP:

The Program on the Economic Evaluation of Medical Technology will be offering a new 3-1/2 day short course on advanced pharmacoeconomics in late Spring 1997.