

John A. Nyman, PhD

### **Overview: The Basics**

conomic evaluations are defined as "the comparative analysis of alternative courses of action in terms of both their costs and consequences."<sup>1</sup> Consequences are measures of the effectiveness of an intervention or treatment. They sometimes are positive, such as extended life spans, and sometimes negative, such as adverse events or increased pain. However, negative consequences are not costs, which are the real resources used in production of the outcomes. These resources are evaluated at their opportunity cost (ie, the value of what those same resources could have produced in their next most valuable use). If markets are competitive, the market price would represent the opportunity cost. Costs sometimes are negative if some resources are saved.

The main statistic used in economic evaluation is the incremental cost-effectiveness ratio (ICER). The ICER tells one the cost (C) of the resources needed to obtain one unit of outcome or effectiveness (E) derived from implementing treatment 2 instead of treatment 1:

$$ICER=(C_2-C_1)/(E_2-E_1)=\Delta C/\Delta E$$

 $\Delta$ =change

ICERs are usually positive ratios because additional costs are needed to obtain effectiveness gains. As such, the ICER often is interpreted as the price of an additional unit of effectiveness obtained by treatment 2 compared with treatment 1.

Economic evaluations make formal the same sort of decisions that consumers make every day. For example, consumers might face two alternative medical treatments—treatment 1 extends life expectancy (LE) by 3 years and treatment 2 extends LE by 5 years. Treatment 1 costs \$40,000 and treatment 2 costs \$120,000. Consumers would want to know what they get for the extra cost of treatment 2. To determine this in everyday life, consumers would implicitly calculate the same ICER:

$$\label{eq:CER} \begin{split} & |CER=(C_2-C_1)/(E_2-E_1) \\ & |CER=(\$120,000-\$40,000)/(5-3 \ years) \\ & |CER=\$80,000/2 \ years \ of \ life \ expectancy \\ & |CER=\$40,000/year \ of \ life \ expectancy \ by \ using \ treatment \ 2 \ instead \ of \ treatment \ 1 \end{split}$$

Consumers would then use this price in deciding whether to use intervention 2 by asking, "Is 1 year of life expectancy worth \$40,000?" If it is, consumers would use treatment 2 instead of treatment 1. In a formal economic evaluation, one would likely compare this ICER price to some benchmark value of a life-year gained or, more likely, value of a quality-adjusted life-year (QALY) gained.

## Four Types of Economic Evaluations

The four types of economic evaluations that are commonly used to facilitate decision making in healthcare resource allocation are cost analysis (CA), cost-effective analysis (CEA), cost-benefit analysis (CBA), and cost-utility analysis (CUA) (Table).

#### Table. Economic Evaluations



B=-benefit

### **Cost Analysis**

Instead of using the entire ICER, the numerator alone is calculated in CA:



A CA is done when it is suspected that the intervention is so effective as to reduce costs in the long run. The change in costs ( $\Delta$ C) includes two components: 1) the additional cost of new treatment 2 itself, and 2) the resources saved downstream because the new treatment 2 is more effective than the old treatment 1 at treating the disease in question. For example, even though the cost of a nutritional supplement is perhaps greater than the cost of standard care, it may reduce morbidity so much that it saves costs over time.

CAs are true economic evaluations because effectiveness is captured in the impact of the intervention on resource use (ie, in the costs). Empirically, they are more difficult because of the econometric challenges of estimating the reduction in costs caused by the intervention using observational data. Theoretically, they are often more controversial because of the lack of standardization regarding what costs to include. Return on investment (ROI) analyses, cost of illness (COI) analyses, and budget impact analyses (BIAs) are the three types of CAs used in practice today.



ROI analyses often are done by firms that want to determine whether purchasing an intervention reduces their costs in the long run. In ROIs, the result often is expressed in savings per dollar expended on the intervention. For example, the University of Minnesota wanted to know if the health promotion program it purchased made employees so much healthier that it saved on healthcare costs in the long run. It was discovered that by the end of the 3rd year, the healthcare cost savings from disease management alone were sufficient to generate a positive ROI of 1.76.<sup>2</sup>

COI analyses are intended to calculate the total cost of a disease to society. Originally, COIs were used to measure the relative importance of various diseases, but now they often are used to determine the economy-wide savings from adopting some intervention. The lack of standardization as to what costs to include makes the findings of COIs vulnerable to manipulation.

BIAs do the same thing as COIs, but concentrate on the costs incurred by a certain payer, rather than society. For example, a recent BIA estimated the total cost saving to the Dutch healthcare system if nutritional supplements were used to treat disease-related malnutrition in the elderly. Overall costs of care were reduced by nearly 20%.<sup>3</sup>

### **Cost-Effectiveness Analysis**

In CEA, the entire ICER is calculated:

 $ICER=(C_2-C_1)/(E_2-E_1)=\Delta C/\Delta E$ 

Costs are in currency units (eg, dollars), but change in effectiveness is in natural units, such as number of infections, systolic blood pressure level, low-density lipoprotein cholesterol level, and so on. Sometimes, these measures are not very meaningful because the reader does not have an appreciation for the natural units used in the analysis. For example, a CEA might find that a nutritional supplement improved glycated hemoglobin (A1c) levels in patients with diabetes by a certain amount for a certain additional cost. This is an important finding, but the number of QALYs that are saved is perhaps more meaningful, so many CEAs also conduct a modeling study to translate the intermediate end points into final end points.

One standard modeling approach is to use a Markov simulation to extrapolate beyond the available data. For example, a Markov model would take the improvement in A1c generated by the nutritional supplement and convert it into its impact on reducing the likelihood of a myocardial infarction and perhaps

other adverse events. The Markov model might run for a certain number of years to determine the reduction in deaths and morbidity from using the supplement compared to not using it. Mortality and morbidity improvements could be combined in a single metric, QALYs, and placed in the denominator of the ICER. This was the approach taken by Randolph et al that estimated the cost-effectiveness of a diabetes-specific nutritional supplement.<sup>4</sup>

### **Cost-Benefit Analysis**

In a CBA, cost and effectiveness are both expressed in currency units. When effectiveness is evaluated in currency units (dollars), it becomes a benefit. For instance, one measure that is employed in CBAs is a benefit-cost ratio (BCR):

 $BCR = (B_2 - B_1)/(C_2 - C_1)$ 

CBAs are comprehensive because it is possible to convert all effects of an intervention into currency and add them together, but attaching dollar values to the health effects is often difficult. Health effects are not marketed, so a market price is not available. Economic theory says that this value is measured by the consumer's willingness to pay for it, but difficulties arise in determining what someone would pay for health improvements, such as reduction in blood pressure or A1c, so economic evaluations have tended to use QALYs to aggregate health effects instead.

### **Cost-Utility Analysis**

When QALYs are used to measure effectiveness, the economic evaluation is called a CUA and the ICER becomes:

 $ICER=(C_2-C_1)/(QALY_2-QALY_1)=\Delta C/\Delta QALY$ 

QALYs are years of life weighted by quality of life (QOL). QOL is a measure of morbidity on a scale of 0 to 1, where QOL=1 is the weight given to perfect health and QOL=0 is the weight given to a health state that is as bad as death. As a result, QALYs are able to combine mortality and morbidity into a single measure.

In 1993, the US Public Health Service convened a panel of experts in economic evaluations and charged them "with assessing the current state-of-the-science of the field [of economic evaluation] and with providing recommendations for conduct of studies in order to improve their quality and encourage their comparability."<sup>5</sup> This



Washington Panel reviewed the various types of analyses and recommended the use of CUA in economic evaluations in the United States. The Washington Panel then set recommendations for standardizing how to conduct CUAs in the United States.

### Policy: United States and the Rest of the World

Many countries have some form of national insurance system. The insurance administration for these countries often desires to serve as a responsible and accountable purchaser. To do so, many countries have established standards for economic evaluations and often also have dedicated agencies to review economic evaluations for coverage of new technologies. It appears that these efforts are most developed in countries such as the United Kingdom and Australia. For example, the rules for conducting a CUA in the United Kingdom are spelled out in the National Institute for Clinical Excellence (NICE) Guide to Methods of Technology Appraisal, June 2008.<sup>6</sup>

Other countries have policies for economic evaluations, but not all as developed as those in the United Kingdom. These policies differ from country to country. These methodological differences can lead to different conclusions regarding the cost-effectiveness of an intervention. For example, some countries recommend including all indirect costs (transportation costs, costs of productivity lost when ill, and informal care costs), while others only want to include indirect costs if they actually are paid for by the national health insurance system. As a result, the ICERs of effective interventions usually are larger (ie, less cost-effective) in the latter countries.

The current United States public policy toward economic evaluations is outlined in the Affordable Care Act (ACA) of 2010. The ACA established a Patient-Centered Outcomes Research Institute (PCORI) to promote the making of informed health decisions and to disseminate scientific information. However, with regard to coverage decisions, PCORI is constrained compared to its counterparts abroad. It appears that this is largely the result of the current hyper-political environment in the United States. For example, death panels are seen by many as the logical conclusion of some efforts to rationalize the system.

The ACA prohibits PCORI from basing coverage or reimbursement policies for public or private insurers on economic evaluations. According to the ACA, the Secretary of Health and Human Services cannot "deny coverage of items or services…solely on the basis of comparative clinical effectiveness research…."

Also, PCORI "shall not develop or employ a dollars-per-quality adjusted life year (or similar measure that discounts the value of a life because of an individual's disability) as a threshold to establish what type of healthcare is cost-effective or recommended...." Furthermore, the Secretary cannot "utilize such an adjusted life year (or such a similar measure) as a threshold to determine coverage, reimbursement, or incentive programs" under Medicare. Still, the ACA has provisions that would make economic evaluations useful, if not necessary.

Private policy toward economic evaluations in the United States is harder to characterize. Many health plans are reluctant to use economic evaluations to determine coverage because of fear of lawsuits.<sup>7,8</sup> The inclination to use economic evaluations varies from plan to plan, and from state to state. For example, the State of Washington's Health Care Authority Health Technology Assessment established coverage recommendations based on both costs and effectiveness, and recently voted not to pay for a continuous glucose monitor because of the lack of evidence of its cost-effectiveness. Other states are considering adopting similar criteria.<sup>9</sup>

### Four Uses of Economic Evaluations

Economic evaluations are used to address at least four types of problems (reviewed next).

#### **Budget Allocation**

The budget allocation problem arises when a government health agency has a fixed healthcare budget that it wants to allocate most efficiently. For example, in the 1990s, the State of Oregon's Medicaid program had a certain limited budget. It could not cover all the care needed by Oregon's Medicaid-eligible population, so it used economic evaluations to determine how it should spend the money. Oregon found the CUA ICERs for all the various healthcare procedures and listed them in order in a league table, with the procedures with the smallest ICERs listed first. Oregon intended to fund procedures in the order of the ICERs until the Medicaid budget was exhausted, an approach that would maximize the number of QALYs gained from the fixed Medicaid budget.

However, this approach was never implemented in Oregon. It was challenged in court because it did not take into account the fact that people with permanent disabilities could not make the same gains in QOL as individuals without disabilities. Therefore, the CUA solution to the budget allocation problem was biased against treatments for Americans with disabilities.



#### Marketing Tool

Economic evaluations also are useful for showing that an intervention saves money. Such a finding is persuasive as long as the long-term savings accrue to the firm that adopts the intervention. Economic evaluations are also helpful in determining at what price a new product saves money compared to a less-effective competing alternative. If a CUA is used, economic evaluation is useful to determine at what price per unit of the treatment the new treatment produces additional QALYs at a cost that is less than the value of a QALY.

#### Coverage

In the United Kingdom, NICE requires a CUA for any new technology considered for use under the National Health Service. Other countries have similar requirements. In the United States, economic evaluations generally are not used to determine coverage. Since 2010, the ACA has prohibited the use of economic evaluations for coverage decisions.

Yet provisions of the ACA run counter to this prohibition. One of these is the ACA directive that acceptable health plans cover essential health benefits (EHBs). EHBs initially were defined by the Institute of Medicine (IOM) as the premium paid by the typical small employer plan—a dollar amount. If defined as a dollar amount, economic evaluations would become useful in determining the most efficient healthcare procedures to cover in the EHB package. Recently, however, the Obama Administration turned the definition of EHBs over to the individual states. However, given the IOM's precedent, some states may use economic evaluations in this process.

The ACA also established a tax on high-premium ("Cadillac") health insurance policies. Any insurance policy with a premium exceeding \$10,200 for an individual or \$27,500 for a family will result in a tax for the excess at a 40% rate, effective in 2018. By 2018, the premiums of many firms will exceed these thresholds. For example, the University of Minnesota estimates that if costs grow at an 8% rate, it will need to pay \$8.9 million in "Cadillac" plan taxes.<sup>10</sup> Thus, many employers will seek to substitute lower cost interventions for higher cost ones. If so, economic evaluations would help in finding the cost-saving healthcare interventions.

While the ACA prohibits use of economic evaluations in determining coverage, it does not preclude the use of economic evaluations in determining cost sharing. Value-based insurance design encourages some types of medical interventions because they save costs or are cost-effective.<sup>11</sup> This care should not be subject to cost-sharing. Indeed, some interventions may result in such a cost-saving that

insurers might even pay individuals to use them. The first step in identifying these cost-saving interventions is an economic evaluation.

#### **Medical Guidelines**

Medical guidelines gained momentum in the United States with the passage of the Omnibus Budget Reconciliation Act of 1989. The theory was that once practice guidelines are determined, it would become possible to eliminate ineffective procedures and thus reduce Medicare costs. Although guidelines are based primarily on effectiveness, some are based on cost-effectiveness. This is especially true of stepped-care guidelines that attempt low-cost interventions first, then move on to more expensive ones. This is another potential use for economic evaluation.

### Conclusions

Economic evaluations represent a policy tool that is useful in determining the price of obtaining a health improvement by using a certain intervention compared to an alternative one. The economic evaluations that are often most useful—CAs—are also often the most difficult to perform and have the least standardization. Many countries rely on economic evaluations to make coverage decisions for their national health systems.

However, the United States has an ambivalent official attitude toward economic evaluations. On the one hand, the government-recommended form of economic evaluations—CUAs—is prohibited by the ACA, but on the other, the results of an economic evaluation would prove helpful in implementing some of the provisions of the act. The future of economic evaluations at the policy level in the United States is unclear.

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