Chapter X

Cost Effectiveness Analyses As Applied to ESRD[1]

As described above in Chapter I, a new goal of the USRDS is to conduct cost analyses and cost effectiveness analyses.1 This additional goal is an application of economic principles to aid in decision making in health care. Decisions, by definition, represent the exercising of choice among alternatives. If there is no choice, there is no decision.

Decisions are made at all levels of health care, from the allocation of resources to research, to clinical alternatives in treating patients, to decisions as to whether an insurer should or should not pay for a particular service. Decisions are made at the national level, the state level, in the dialysis clinic and in the patient's home. Decisions are made by patients, by physicians, by other care givers, by public and private administrators, and by elected officials. Analyses of the associated costs and outcomes for these alternatives have been developed to help rationalize this decision process.

Economics has been described as the science of choice, i.e. the selection among options or alternatives when facing a finite limit of resources. Cost analyses in ESRD can be defined as almost any decision analysis in which the use of finite resources, such as money or limited donor organs for transplantation, is applicable.

This chapter will provide a primer on cost effectiveness with a general application to ESRD. The examples that are given focus on the issues involved in the choice of modality for treating ESRD such as hemodialysis, CAPD and kidney transplantation. The methodology, however, applies to practically all clinical decisions involved in the treatment of ESRD patients.

Cost and Outcomes of Alternative Health Care Decisions

As described above, cost analyses are usually based on comparisons among specified alternative therapies, although one form of analysis described below (cost benefit analysis) can guide a

[1] Cost effectiveness as used in this report is a generic term that applies to all cost analyses of alternative health outcomes. Unfortunately, one specific form of cost and outcome analysis is also called cost effectiveness. To distinguish between the generic use and the particular use, an italicized *cost effectiveness* will refer to the particular form of cost and outcomes analysis.

judgment about one program evaluated on its own merits.

There are three forms of analysis that might be used for a cost analysis of medical alternatives. They are *cost effectiveness analysis* (CEA), cost utility analysis (CUA) and cost benefit analysis (CBA).

CEA is most often used when all relevant outputs are in the same units, usually deaths averted or life years saved. Cost utility analysis is often used when multidimensional outputs can be converted to a single output measure, usually quality adjusted life years (QALY). The third method, cost benefit analysis, is used when the value of all outputs and costs can be measured in dollars.

This division between *cost effectiveness* and cost utility analysis follows the distinctions made by Torrance (Torrance). Work by Weinstein and Stason (Weinstein) does not follow this distinction and uses the term *cost effectiveness* to include both *cost effectiveness* and cost utility analyses. More recent work by Phelps and Mushlin (Phelps) reports an equivalence between *cost effectiveness* and cost benefit analysis.

Viewpoint or Perspective

One of the first choices in a cost and outcomes analysis is to define whose costs are an issue. For example, the costs facing an ESRD patient insured under the Medicare program are different than the costs facing the administration of the Medicare program which funds most care for ESRD patients. Depending on the evaluation, both costs may be relevant. The costs analyzed then depend on the decisions to be made and on the persons or institutions affected by the costs.

It is often assumed in cost effectiveness analyses that decisions made by public bodies have the viewpoint of society as a whole. Although the accuracy of this assumption has never been verified, the public viewpoint is usually assumed when conducting cost effectiveness analyses of medical care programs, particularly of public funded programs.

The cost to the federal government of most decisions related to the treatment of kidney failure will certainly be an issue. Since the patient is the decision maker that decides to accept or not accept a particular clinical decision, the costs faced by patients are usually an issue as well. State governments also face considerable costs for the ESRD program since many of the patients with ESRD are eligible for Medicaid, a federal and state program that provides health insurance for the poor. Many private insurers also face costs for kidney failure patients through coinsurance (patient obligations) and other provisions such as employer group health insurance, which is often the primary insurer for the first year and a half of renal failure. (Medicare is the secondary insurer for Medicare eligible patients in such cases.)

Part of the decision process in choosing a viewpoint is the type of costs to include. The direct costs of care are the costs for direct medical services, but indirect costs such as lost production from work should be considered, depending on the viewpoint.

It is important in cost and outcome analyses to use the same viewpoint consistently across all parts of the analysis. For example, if an analysis from a societal perspective used the incremental (marginal) cost of care for inpatient services to evaluate cost, then the same measure of cost should be used for outpatient drugs. Keep the viewpoint consistent.

Measuring Benefits

Another major issue is how to measure outcomes or benefits. What value is placed on life extended, for example, if modality A has longer survival than modality B?

Generally, there are three ways to value benefits. **First** is the measurement of life extended in simple terms such as days or years. The benefit is frequently called life years gained. One limitation of this approach is that it does not give consideration to other aspects of life, primarily the quality of life. This limitation has led to the **second** method of valuing outcomes, the quality adjusted life year (QALY, or QUALs), which is probably the most commonly used method of assessment currently employed in cost effectiveness analyses. This methodology is based on measuring a set of states of the world in which one can exist (e.g., able to climb steps) and then using a set of weights, derived from either this population or another, to aggregate across these states of the world to obtain an "adjuster," such that the years in different states are comparable.

An adjuster which is close to one is usually considered to represent a healthy state. An adjuster near zero is near death. For example, one year with CAPD therapy might yield a value of 0.60 of a normal year, i.e., 40 percent loss of normal quality of life. If survival averaged six years, then the effectiveness of this therapy would be 3.6 QUALs (0.6 x 6). If, in comparison, one year with a functioning kidney transplant had a value of 0.80 for a quality adjusted life year, and if survival averaged 10 years, the QUALs for this modality would be 8.0 (10 x 0.8). The comparison would then be between 3.6 and 8.0 QUALs.

The most common method of analysis to estimate these “adjusters” is to use weights from a general population survey (Bush). More recent work on methods, such as the Medical Outcomes Studies, have developed weights based on other surveys of the general population (Ware). One of the more recent major advances involves the development of the SF-36 questionnaire, which can be readily used to evaluate several indicators of quality of life using standard indexes. This questionnaire has been used in evaluating quality of life in ESRD and in other areas of health care.

An issue of some interest and relevant to therapy choices in ESRD is whether a set of weights (values) derived from ESRD patients might be relevant for cost utility analysis. One of the frequent findings regarding ESRD patient quality of life is that ESRD patients subjectively do not find their lives as bleak as judged by others (Evans).

A **third** method of evaluation of outcomes is the “willingness to pay,” i.e., how would a consumer value the state described? In the current example, the issue is how a consumer would value life with CAPD compared to life with a kidney transplant. These valuations have usually been measured in monetary terms.

Historically it has been accepted wisdom that it is very difficult if not impossible to implement such measures of monetary value in a practical setting. But recent work by two Swedish researchers (Johannesson and Jönsson, 1991) have presented evidence that one can develop an empirical basis on which to value alternative outcomes in monetary units for health care alternatives. Their methodology is called **contingent valuation** and is a popular and frequently employed technique of valuation in environmental economics. These techniques, if successfully applied in health care, give new life and meaning to both the willingness to pay and cost benefit analysis.

Adjustments for Intertemporal Events: Discounting

A quickly recognized fact of the primary alternatives for the treatment of patients with ESRD is that one alternative, transplantation, presents a situation where the up-front costs are quite large compared to the yearly "maintenance" costs. Similarly, the benefits are derived over a relatively long time stream.

Dialysis, whether hemodialysis or peritoneal dialysis, presents a stream of reasonably stable costs that in any one year are generally lower than the initial cost of transplantation. Converting these alternative cost and benefit streams to a common time period for comparison is a normal part of cost and outcomes analysis. This process of conversion is called discounting.

Discounting of costs follows the general formula of:

$$\text{Discounted Cost} = \sum_t \frac{\$_t}{(1+r)^t}$$

where time (t) is from start of study, t_o; to death or censor t_d; r is the appropriate discount rate; and $ is the cost in period t. In effect, discounting of costs implies that a dollar today is worth more than a dollar next year, even if there were no inflation.

This appreciation in the value of resources over time is due to differences associated with inflation-free dollars and reflects how society prefers consumption today vs. investment today and consumption some time in the future.

Whether to discount outcomes and how this should be done is more controversial than is the case for costs. We will discuss whether to discount first.

While the concept of discounting is easy to describe, it is often unpopular and misunderstood in medical circles (particularly the adjustment for the length and quality of life). In essence, the concept implies, for example, that for a thirty year old patient, a year of life of given quality is valued more at age 35 than would an additional year when the same patient reaches age 60. This does not mean, however, that a year of life to a person 35 years of age is worth more than a year of life for another person who is 65. The discounting process is only used to move all comparisons to the same time period. For example, it is not used to make comparisons among persons of different ages.

One of the original manuscripts arguing for discounting of outcomes is by Keeler and Cretin (Keeler). The basis of their argument is that projects in the public interest by rational decision makers will never be justified if one does not discount benefits. Johannesson and Jönnson (Johannesson and Jönnson 1991, Health Policy 17) describe this conceptual difficulty as "inconsistencies in the analysis." Essentially, the argument is that without discounting of the benefits, there will never be a situation to decide to go ahead with a social investment. If future benefits are as good as current benefits, i.e. a situation with no discounting, then a rational decision maker would always save the money now. Since this money will be worth more in the future, a future purchase can buy more benefits. Given that the real rate of interest is always positive, the rational decision to invest will never be made. The reason, of course, is because then it will always be better to invest in future projects. If future benefits are of the same value as current benefits, public welfare is improved by buying more benefits in the future. Only by discounting benefits, Keeler and Cretin argue, does the rational decision maker find the rationality to make an investment.

Some argue that benefits should not be discounted at all, while others argue for discounting benefits at a lower rate than the rate used to discount cost (Johanneson 1992; Olson; Parsonage). However, the mainstream research methods argue for the discounting of benefits.

Determination of the proper discount rate is subject to considerable debate. Many have argued that a rate that approximates the long-term real interest rate of between 3 and 5 percent is the correct choice. This real rate of discount cited here is without inflation (Olson; Johanneson 1992).

A safe and conventional alternative is to perform the analysis using two or more discount rates and then to allow the final users of the analysis to choose, based on their perceived values. Official federal government policy (Omnibus Reconciliation Act) has been to use 10 percent for discounting. A 10 percent discount rate is considered high by most standards. Use of this rate implies that future costs, savings and benefits are worth less than if lower discount rates are used. A 10 percent discount rule is a good way to reduce government spending. Much government investment is, like private investment, typified by large up-front costs and delayed benefits. A typical example might be a dam built by the Army Corps of Engineers, but there are strong similarities to a decision to invest in a kidney transplant. When delayed benefits are discounted by a comparatively high discount rate, the resulting discounted benefits are relatively small. Similarly, if one were discounting future year savings due to averted dialysis, these savings too would be smaller. For example, an annual per patient cost of $46,000 for ESRD five years in the future is only $29,000 in present value terms if a discount rate of 10 percent is used; the cost is $36,000 if 5 percent is used.

In summary, there is a general consensus in the current mainstream literature on cost effectiveness analyses that benefits should be discounted as well as costs. The issue of precisely how to discount costs and benefits is not without controversy. However, the discount rate that is selected should be applied to both costs and benefits to ensure that decisions are based on future costs and benefits that are comparable.

Choice of a Form of Analysis

Three potential forms of analysis are compared in Table X-I. The first is the *cost effectiveness analysis* (CEA) briefly described above. *Cost effectiveness analysis* would be most applicable in a situation in which both modality A and modality B have only one relevant outcome measure, e.g., years of life saved. In other words, the CEA form of analysis only considers the cost per year of life saved with no consideration of other outcomes such as an intrinsic value to life or changes in the quality of life. If the quality of life is not an issue, either because of the goals of the decision maker or because there are in fact no differences, then CEA may be the appropriate choice.

The cost utility analysis (CUA, summarized in column 2 of Table X-1) has the advantage of considering both the quality and quantity of the change in outcomes. The quality of life is important, if as previously mentioned,

Potential Forms of Analysis of Costs and Outcomes in the ESRD Program[1]

	Alternative Forms of Analysis		
Aspect of Analysis Form	***Cost-Effectiveness[2] (CEA)***	**Cost-Utility[2] (CUA)**	**Cost-Benefit (CBA)**
Units of measurement	Cost-Effectiveness Ratio, e.g., $ per life-year gained	Cost-Utility Ratio, e.g., $ per quality-adjusted life-year gained	Benefits and opportunity costs are measured in $'s.
Most useful situations	Comparing alternative programs whose effects are in same units, e.g., alternative drugs to control hypertension where output is years of life saved	1. When quality of life is an important outcome 2. When morbidity and mortality are affected	Potentially can examine single programs or possibly a single clinical choice. Can also evaluate among alternatives.
Comments	Not useful when: 1. Analyzing a single program 2. Disparate alternatives, e.g., home dialysis and care of frail elderly	Probably the most common approach today. Not useful when: 1. Analyzing a single program	1. Can be difficult to measure outcomes in dollars. 2. Contingent valuation shows promise of valuing outcomes in $'s.
Decision criteria for comparing between alternatives	1. If effectiveness is the same, choose least cost 2. If effectiveness is different, compare incremental cost/year with external standard	1. If effectiveness is the same, choose least cost 2. If effectiveness is different, compare incremental cost/year with external standard	Choose programs whose benefits exceed their opportunity cost

[1] Much of this material is drawn from Torrance, 1986; and Johannesson and Jönsson, 1991.
[2] Some authors treat these alternatives as one form of analysis.

Table X-1

the overall style and enjoyment of life from diagnosis to death is different under the two alternative forms of treatment.

The decision criteria depend on whether the effectiveness is the same or not. If both alternatives are equally effective, then the decision criteria depend only on a comparison of the discounted costs. If the effectiveness is different between the two alternatives, then the decision criteria depend on the incremental cost utility ratio.

The third form of analysis shown in Table X-1 is cost benefit analysis (CBA), which measures all costs and benefits in dollar terms. Costs should ideally be measured in social opportunity cost, i.e. value of the goods and services which the health care resources could produce outside the health sector if they were so employed (Johannesson and Jönsson 1991).

The decision criteria under CBA is simple: select any choice for which the benefits exceed the opportunity costs of production.

The CBA form of analysis has generally been limited by the difficulty of measuring benefits in dollar terms. As discussed above, some new developments in Sweden which employ techniques readily used in environmental economics suggest these problems may not be as insurmountable as previously thought.

DECISION CRITERIA

Same Effectiveness

To choose among a set of alternatives, such as hemodialysis and kidney transplantation, a set of decision criteria is needed. This is true for both *cost effectiveness analysis* and cost utility analysis. In both forms of analysis, the criteria are identical. The difference in the current case is whether the effectiveness is measured in added years of life (for CEA) or added quality adjusted life years (for CUA).

The first criterion is the relative effectiveness of the two alternatives to be compared. For example, suppose that both alternative therapies being compared save the same number of discounted life years (or, as appropriate, discounted QUALs). Then the decision criterion is simply the relative cost of each alternative, where all costs have been discounted to the same period. If one alternative yields lower costs than another, such a comparison situation is considered dominant in that one therapy is clearly preferred (Wong). If, for example, the choices were X and Z and X and Z had similar survival while X had lower costs over the lifetime of a patient, then X would dominate Z.

Different Effectiveness

Similar to the dominant case above, if the costs are the same, but the effectiveness is different, then the choice is simple: chose the more effective alternative. This would clearly be a case of dominance (Wong).

When the effectiveness and costs of the therapies being compared are both different, then the criteria for decision are more complicated. Suppose, for example, that one alternative leads on average five added life years, while the other alternative leads to seven added life years. The criteria for choosing then depend on the incremental cost for the difference in life years between the two alternatives and some external criteria of what an added life year is worth to the decision maker. In other words, the comparison is not absolute or dominant but depends on some external criteria of what the added years are "worth." Some hypothetical examples are shown to better illustrate this situation.

Consider the example data shown in Table X-2 and the examples of actual comparisons shown in Table X-3. Assume that the accepted therapy is A, for example, dialysis. How would the above criteria apply to the examples of B through H in Table X-2? In case A vs. C, where the effectiveness is the same (i.e. 5 years), the decision criteria are solely based on costs. C is clearly preferred (dominant), since one can obtain more years of life for fewer lifetime dollars. The incremental cost per year for the additional years of life is negative.

In comparing B vs. H, average costs are lower for H compared to B. However, while the lifetime costs are higher under B, the years of life saved are higher too. For an additional $135,000 B will provide an additional 3 years of life gained. Which alternative should be chosen? This is clearly a case where one has to resort to some external standard of judgment. Does society value the additional years of life gained by an amount equal to or greater than the increased costs? Typically, the decision is made by comparison to alternative processes by which society makes life

Costs and Added Life Years for Hypothetical Alternatives for ESRD Patients[1]

Alternative	Total Lifetime Cost (1,000s)	Added Life Years (Effectiveness)	Average Cost per Life Year (1000's)
A	$225	5	$45.0
B	270	7	38.6
C	175	5	35.0
D	225	7	32.1
E	200	4	50.0
F	275	4	68.8
G	325	7	46.4
H	135	4	33.8

[1]*Costs and added life years are discounted.*

Table X-2

Decision Criteria for Hypothetical Alternatives in ESRD: Comparisons of Different Effectiveness with Assumed Standard

Comparison		Cost/Patient (1000's) Total	Avg/Year	Effectiveness (years)[1]	Incremental Cost/Year (1000's)	Cost Effectiveness Decision
A vs. D	A	$225	$45.0	5	< $0 with D	Choose D
	D	225	32.1	7		
A vs. E	A	225	45.0	5	$25 with A	External
	E	200	50.0	4		Standard
A vs. C	A	225	45.0	5	< $0 with C	Choose C
	C	175	35.0	5		
B vs. H	B	270	38.6	7	$45 with B	External
	H	135	33.8	4		Standard

Table X-3

saving decisions. For example, comparisons might be made to other forms of health care, or other areas such as highway safety.

The comparison between B and H in Table X-3 illustrates a case where a different and incorrect decision might be made by focusing on average costs rather than incremental costs**. A comparison of average costs between alternative therapies does not effectively consider any potential difference in outcomes. The criterion should therefore be the incremental cost per added life year where the effectiveness of existing alternatives is different.**

Consider other examples depicted in Table X-3 wherein the effectiveness is different and the costs are also different. In comparing A and D, both have the same total lifetime cost per patient. Therapy D, however, has a higher effectiveness in that the average patient has an added seven years of life. As a result, the average cost per year for D is less than for A. But the criteria described above indicate that the relevant criterion is the incremental cost for the added years, and in this case there is no added cost for two extra life years. Clearly, D would be preferred to A (D dominates A). In this case, the choice is clear since D offers more added years of life for the same cost.

In the comparison of A vs. E, alternative E has higher total costs but more years are saved. How does one then compare these choices? For the incremental $25,000, the patient lives one more year for an incremental cost per year of $25,000. Whether this extra year is "worth" the extra costs cannot be decided by comparisons within this model. Such decisions depend on an external standard. It is not easy to derive such standards, but comparisons to other societal choices involving life saving decisions are frequently used.

Summary

In some situations cost effectiveness comparisons indicate a clear choice among alternatives, while in other situations an external standard needs to be applied. A clear choice can be made when one therapy results in both more added years of life and lower lifetime costs, or in both the same added years of life and lower lifetime costs. In this case, there is no incremental cost associated with the added survival. A different scenario results when one therapy has better survival, but is associated with higher lifetime costs than the comparison group. In such a case, an external standard must be applied to determine whether the positive incremental cost is "worth" the added survival from society's viewpoint.

The discussion and examples in this chapter have focused on the larger choices of modality for treatment of chronic renal failure with outcome measures such as morbidity and mortality. Obviously, there are numerous other clinical choices to be made and other outcomes to be measured in the treatment of patients with ESRD. For example, the choice of vascular access devices for dialysis patients could be readily analyzed within a cost effectiveness framework. Similar analyses could be performed for choice of dialyzer membrane, and the choice of germicides and techniques used to disinfect dialyzers for reuse.

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Endnotes

[1] This primer draws on many sources. The work of Weinstein and Stason (1977), Torrance (1986), and Johannesson and Jönsson (1991) deserve special mention.