

doctor to certify both therapeutic benefit and consent) for treatments that “give rise to special concern.” The secretary of state has chosen not to include goserelin in this category in the revised code of practice coming into effect on 1 November. Had she done so and chemical castration been recommended to Mr K as a treatment for mental disorder, legal recognition of special concern might have approximated such treatment to orchidectomy. But goserelin has not been included, and, furthermore, neither treatment option for Mr K is apparently directed to mental disorder. Goserelin is thus a far cry from invasive, irreversible orchidectomy. This only adds to the dilemma of patient and doctors alike.

Application might be made for a judicial declaration that orchidectomy is lawful in this case. The casuistry of such litigation depends on medical opinion of what is appropriate. The best interests approach is often taken without specifying which interests are the best or who

decides.⁴ Despite its limitations the best interests approach is in any case better suited to decisions concerning treatment of incapable patients than it is to decisions denying treatment to a patient in full possession of his mental faculties.⁵

Legislation and case law offer analogies both for and against performing orchidectomy. The decision in the case of Mr K could be made by a judge, by an ethics committee, or simply from an informal second opinion. Mr K, however, might justifiably expect his problem to be resolved not by asking why his desired operation should be performed but rather by asking why it should not if his autonomy and desire not to reoffend are to be respected.

1 *Devi v West Midlands Regional Health Authority*; Court of Appeal 7 May 1980.
 2 Dworkin G. The law relating to organ transplantation (1970) 33 MLR 353, 355.
 3 *R v Mental Health Act Commission, ex parte W* (1988). *Times*, 27 May.
 4 *F v West Berkshire Health Authority* [1990] 2 AC 1.
 5 See Law Commission. *Consultation paper No 129*. London: HMSO, 1993.

Economic Evaluation and Health Care

Cost-effectiveness analysis

Ray Robinson

This is the third in a series of articles that describes the ways in which methods of economic evaluation may be used to assess the economic costs and consequences associated with different forms of health care intervention

When different health care interventions are not expected to produce the same outcomes both the costs and the consequences of the options need to be assessed. This can be done by cost-effectiveness analysis, whereby the costs are compared with outcomes measured in natural units—for example, per life saved, per life year gained, and per pain or symptom free day. Many cost-effective analyses rely on existing published studies for effectiveness data as it is often too costly or time consuming to collect data on cost and effectiveness during a clinical trial. Where there is uncertainty about the costs and effectiveness of procedures sensitivity analysis can be used, which examines the sensitivity of the results to alternative assumptions about key variables. In this article Ray Robinson describes these methods of analysis and discusses possibilities for how the benefits of alternative interventions should be valued.

If the outcomes of alternative procedures or programmes under review are the same, or very similar, then attention can focus upon the costs in order to identify the least cost option. The method of evaluation for this—cost-minimisation analysis—was described in last week’s article. If, however, the outcomes are not expected to be the same, then both the costs and consequences of alternative options need to be considered. Cost-effectiveness analysis is one method of economic evaluation that allows this to be done.

Measures of effectiveness

In order to carry out a cost-effectiveness analysis it is necessary to have suitable measures of effectiveness. These will depend on the objectives of the particular interventions under review. In all cost-effectiveness analysis, however, measures of effectiveness should be defined in appropriate natural units and, ideally, expressed in a single dimension.

Common measures used in several studies have been “lives saved” and “life years gained.” Thus Boyle and colleagues, in their study of neonatal intensive care of very low birthweight babies, measured effectiveness in terms of mortality rates at the time of discharge of

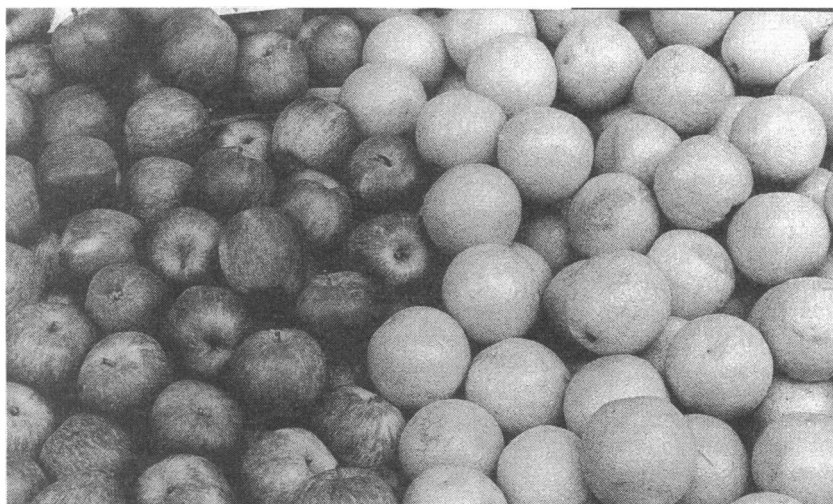
newborn infants from hospital.¹ Their study compared two periods—one before the introduction of neonatal intensive care, and one after its introduction—and measured cost effectiveness in terms of additional costs per life saved. Both Ludbrook² and Churchill and colleagues³ investigated alternative treatments of end stage renal disease and measured their effectiveness in terms of life years gained. A similar measure was adopted by Mandelblatt and Fahs, who reported that the early detection of cervical cancer through Pap tests saved 3.7 years of life per 100 tests and represented a cost of \$2874 per life year saved.⁴ Oster and Epstein also used years of life saved as a measure of effectiveness in their study of antihyperlipaemic therapy in the prevention of coronary heart disease.⁵

Several other measures of effectiveness have been used by different researchers (box). These have included the number of pain or symptom free days resulting from alternative drug regimens in the treatment of duodenal ulcers⁶; the number of patients with a successful diagnosis in the case of alternative diagnostic strategies for deep vein thrombosis⁷; the number of complications avoided in the treatment of Lyme disease after tick bites⁸; and the number of episodes of fever cured and deaths prevented in the treatment of chloroquine resistant malaria in African children.⁹

Most of the above studies express effectiveness in terms of a single dimension and thereby permit direct comparison between alternative procedures in terms of their marginal cost per unit of outcome. Sometimes, however, the alternatives under examination have multiple outcomes. None the less, many of these choices can be dealt with within the cost-effectiveness analysis framework. Thus if one procedure emerges as less costly and of equal or greater effectiveness than all the other options on each dimension of effectiveness, it is clearly the most cost effective option. For example, Percival and Setty, in their comparison of day surgery with overnight inpatient care for cataract surgery, measured outcomes in terms of the number of both operative and postoperative complications, and in terms of visual acuity of patients three to six days and 10 weeks to six months after surgery.¹⁰ Patient satisfaction was also elicited through a questionnaire. As day surgery emerged as the more effective option on

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Assessing multiple choices—
informed decision making or
trying to compare apples and
oranges?

practically all of these effectiveness measures, and was subsequently less costly, the evidence suggests that it is the preferred option.

One argument for carrying out analysis in this way—that is, not always seeking to combine outcome measures into a single unit, is that the variations across a number of dimensions are made clear to decision makers rather than being concealed within an aggregate measure. This can sometimes permit more informed decision making, although it can also result in tortuous attempts to compare apples and oranges.

Obtaining effectiveness data

Data on effectiveness for use in cost-effectiveness analysis may be obtained in a number of ways. Ideally, economic evaluation should be built in alongside clinical trials so that relevant data on costs and effectiveness can be collected at the same time. However, setting up and conducting appropriate trials is often time consuming and expensive. Because of this, many cost-effectiveness analyses rely on the existing medical literature for their data. Unfortunately though, there is often a lack of good epidemiological evidence relating health inputs to outputs, particularly in the case of new technologies, which are commonly the focus of economic evaluation.¹¹ Even when data are available it is important to ensure that they are relevant to the context in which an evaluation is being carried out—for example, do medical and other staff have the same skills and experience as those reported in the studies? Is the same range of medical and surgical equipment available?

If neither specifically designed clinical trials nor the existing published work provide the necessary data in full, an economic evaluation may have to rely on assumptions about clinical evidence.¹² Although this may seem like a dangerous practice, there is often a range of situations in which cost considerations dominate, and so variations in effectiveness are unlikely to alter the preferred option. In such cases, however, the results must be subjected to a range of different assumptions about effectiveness. This can be done by sensitivity analysis.

Uncertainty and sensitivity analysis

RISK AVERSE STRATEGY

In many studies there is a good deal of uncertainty about the costs and effectiveness of different procedures. One way of dealing with this uncertainty is to adopt a risk averse strategy. This is particularly appropriate when comparing a new technology with an established one and entails loading the assumptions against the new technology whenever uncertainty

arises. If the new technology emerges as the preferred option, even after the assumptions have been stacked against it, some confidence can be placed in the results. Such an approach was used by Culyer and Maynard in their study of the relative cost effectiveness of surgery versus the then new option of cimetidine in the treatment of duodenal ulcers.¹³ Despite the systematic loading of assumptions in favour of surgery, they concluded that there was little doubt that drug therapy was more cost effective when the choice was clinically acceptable.

SENSITIVITY ANALYSIS

Another approach to uncertainty about the costs and effectiveness of different procedures is to use sensitivity analysis. This permits the robustness of the results to be tested in light of variations in the values of key variables. Briggs and colleagues identified different forms of sensitivity analysis.¹⁴

Simple sensitivity analysis entails varying one or more of the components of an evaluation to see how it affects the results. For example, Sculpher and colleagues varied both the lengths of inpatient stay and the hotel costs per inpatient day in their study of alternative treatments of menorrhagia.¹⁵

Extreme scenarios is another form of sensitivity analysis. Thus if two treatments are being compared this approach would seek to identify extreme estimates of cost and effectiveness so that the two options can be compared under pessimistic (high cost and low effectiveness) and optimistic (low cost and high effectiveness) assumptions.

Probabilistic sensitivity analysis assigns ranges and distributions to variables, and computer programs are used to select values, at random, from each range and to record the results. The advantage of this approach is that it can simultaneously deal with a large number of variables and indicate the degree of confidence that can be attached to any option.

By using these different methods of sensitivity analysis it is possible to show whether the results of a particular study are robust over a range of assumptions or hinge on the accuracy of particular assumptions. Many studies, however, do not include sensitivity analysis. Thus in a recent audit of 77 articles appearing in American general medical, general surgical, and medical subspecialty journals over the two periods 1978-80 and 1985-7, Udvarhelyi and colleagues found that, despite the frequent citation of limitations in underlying assumptions, sensitivity analysis was applied in only 30 per cent of the studies reviewed.¹⁶

Discounting benefits

In last week's article I argued that costs that are incurred at different points in time need to be "weighted" or discounted to reflect the fact that those which occur in the immediate future are of more importance than those which accrue in the distant future. This raises the question: should the benefits or effects of alternative procedures also be discounted?

There is some disagreement about this among economists. Although the traditional view has been that benefits should be discounted, several economists have argued that this is inappropriate. This stance has recently been given added force by two Department of Health economists who reported that the department and the Treasury have agreed to the desirability of a zero discount rate—that is, there should be no discounting of benefits.¹⁷

The main arguments against discounting benefits are, firstly, that health is not a tradeable resource that can be invested to produce future flows of health, nor does it have a monetary value that can be expected to increase with income over time, and, secondly, there is

Examples of measures of effectiveness

- Cases treated appropriately
- Lives saved
- Life years gained
- Pain or symptom free days
- Cases successfully diagnosed
- Complications avoided

Summary

- Cost-effectiveness analysis is a form of economic evaluation in which the costs of alternative procedures or programmes are compared with outcomes measured in natural units—for example, cost per life year saved, cost per case cured, cost per symptom free day
- Effectiveness data are ideally collected from economic evaluations built in alongside clinical trials. In the absence of dedicated trials researchers need to draw on the existing published work
- Sensitivity analysis should be applied when there is uncertainty about the costs and effectiveness of different procedures. This investigates the extent to which results are sensitive to alternative assumptions about key variables
- There is debate among economists about whether benefit measures should be “time discounted” in the same way as costs. If they are not, whereas costs are, projects with long lasting effects will become relatively more cost effective—for example, maternity services and health promotion. But it is probably too early to recommend this as a standard practice

no evidence to support the opinion that people view future health states less highly than present ones.¹⁸ Against these views other economists argue that activities such as smoking and heavy drinking suggest that people discount the future consequences of their actions. In the words of the eminent welfare economist, A C Pigou, they have “defective telescopic vision.” Moreover, they also argue that certain perverse consequences would follow if costs were discounted and benefits were not. For example, postponing projects indefinitely would become increasingly attractive because their costs would fall as they were shifted further and further into the future, whereas future benefits would continue to be assigned the same value as today, no matter when they occurred.

If a zero discount rate is adopted, the main consequence would be to change the relative cost effectiveness of different procedures. Using a positive discount rate means that projects with long lasting effects receive lower priority. If a positive rate is replaced by a zero rate, procedures such as neonatal care—which lead to benefits over the recipient’s entire future lifetime—will become relatively more cost effective.

In practical terms it is probably true to say that while the case for using a zero discount rate for benefits has powerful intellectual force and may gain empirical support in the future, it is too early to recommend that positive rates are discarded in economic evaluations.

Conclusion

The term cost-effectiveness analysis is sometimes used loosely to refer to all forms of economic evaluation. In its more precise sense, however, it refers to those evaluations that measure the outcomes of alternative procedures or programmes in natural units. Until the early 1980s cost-effectiveness analysis was probably the most commonly used form of economic evaluation. Since then, however, the limitations imposed by one dimensional outcome measures that are specific to particular diseases has led to the development of more general measures of outcome. The next article in this series looks at the way in which cost-utility analysis uses these measures.

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Style Matters

Advertising in medical journals

International Committee of Medical Journal Editors

The following statement was agreed by the International Committee of Medical Journal Editors (the Vancouver Group) at its meeting last month in Chicago.

Most medical journals carry advertising, and advertising generates income for owners of journals, but advertising must not be allowed to influence editorial decisions. Editors must have full responsibility for advertising policy, and readers should be able to distinguish readily between advertising and editorial material. Juxtaposition of editorial and advertising material on the same product or subject should be avoided wherever possible. Finally, editors should

consider for publication all criticisms of advertisements.

Members of the committee are: Suzanne and Robert Fletcher, Kathy Case (*Annals of Internal Medicine*), Laurel Thomas (*Medical Journal of Australia*), Richard Smith, Jane Smith (*British Medical Journal*), George D Lundberg, Richard Glass (*Journal of the American Medical Association*), Robin Fox (*Lancet*), Magne Nylenna (*Tidsskrift for den Norske Laegeforening*), Lois Ann Colaianni (*Index Medicus*), Jerome P Kassirer, Marcia Angell (*New England Journal of Medicine*), Richard G Robinson (*New Zealand Medical Journal*), Bruce P Squires (*Canadian Medical Association Journal*), Linda Hawes Clever (*Western Medical Journal*), Patricia Woolf (Princeton).

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