Some Principles of Cost-Effectiveness Analysis in Dentistry

Summary

The number of economic evaluations in the medical and dental literature is increasing. Cost-effectiveness analyses are useful to inform decision and policy makers about the managerial implications of different treatment policies. Several principles of cost-effectiveness analysis using a critical appraisal of a published economic evaluation in dentistry are reviewed. An improved understanding of the principles behind, and steps involved in the critical appraisal of health economic studies, should improve decision making within the dental community.

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Introduction

Evidence-based medicine is currently en vogue and can be described as the explicit use of best scientific evidence available, in addition to the clinical experience, to make decisions concerning patient care (Sackett et al. 1996). The recent literature indicates that the dental community is beginning to adopt the ideas of evidence-based decision making (Dodson 1997), so-called evidence-based dentistry (Shaw 1997). The application of evidence-based medicine or dentistry requires skills such as i) converting clinical problems into explicit questions, ii) searching the literature efficiently, iii) critically assessing the validity of the selected studies and iv) applying the results to clinical practice. The appraisal of the validity of different types of clinical and health economic studies has been described by Sackett et al. (1997). The criteria by which cost-effectiveness analyses may be assessed are explained in more detail by Drummond et al.

Address for correspondence:

Prof. Dr. C. Marinello, Clinic for Fixed and Removable Prosthodontics and TMJ Disorders, Dental School, University of Basel, Hebelstr. 3, CH-4056 Basel, Switzerland, Tel. ++41 61 267 26 31, Fax ++41 61 267 26 60 Peter P. Sendi, Andrew J. Palmer*, Carlo P. Marinello

Clinic for Fixed and Removable Prosthodontics and TMJ Disorders, Dental School, University of Basel and *Institute for Medical Informatics and Biostatistics, Riehen, Switzerland

(1997a) and O'BRIEN et al. (1997). Some fundamentals are also described as part of an article series on evidence-based medicine for Swiss physicians (SENDI et al. 1998). It should be noted that for an extensive critical appraisal of a cost-effectiveness analysis often more information is needed than is actually available from the published article.

The recently published recommendations of the Panel on cost-effectiveness in health and medicine for the conduct and reporting of cost-effectiveness analyses represent the viewpoint of some US investigators (SIEGEL et al. 1996, WEINSTEIN et al. 1996, RUSSELL et al. 1996). Although some recommendations represent certain improvements in this field, other recommendations were seriously criticized (BROWER et al. 1997, BIRCH & GAFNI 1993, GAFNI & BIRCH 1993). However, these publications mainly address physicians who are exposed to a much higher number of economic evaluations than dentists. In times of scarcity of health care resources, it becomes crucial to develop and apply methods that help to justify, reject or adopt different treatment policies under different circumstances.

In dentistry, the method of cost-effectiveness analysis, and methods for economic evaluations and policy analysis in general, are in their infancy (LIPTON 1991). The number of published cost-effectiveness analyses is limited and an application of strict criteria to assess the validity of those analyses would dramatically reduce the number of methodologically sound studies. The purpose of this article is not to criticize the scarce dental literature in this field, but to expand the understanding of the method of cost-effectiveness analysis within the broader dental audience by illustrating how to critically appraise a cost-effectiveness analysis applied to a dental setting.

Critical assessment

MARYNIUK et al. (1988) performed an economic evaluation comparing the strategies of replacing a failed large amalgam in a posterior tooth with either another amalgam or a crown. Kerschbaum (1997) recommended a careful study of this article. Some basics of cost-effectiveness analysis will be reviewed by critically assessing this article using the criteria suggested by

DRUMMOND et al. (1997a) and O'BRIEN et al. (1997) (Table I). These questions aid in the evaluation of the appropriateness of the methodology and the validity of the results.

Table I Criteria by which a cost-effectiveness analysis may be assessed

- Did the analysis provide a full economic comparison of health care strategies?
- 2. Were the costs and outcomes properly measured and valued?
- 3. Was appropriate allowance made for uncertainty in the analysis?
- 4. Are estimates of costs and outcomes related to the baseline risk in the treatment population?
- 5. What are the incremental costs and outcomes of each strategy?
- 6. Do incremental costs and outcomes differ between subgroups?
- 7. How much does allowance for uncertainty change the results?

1. Did the analysis provide a full economic comparison of health care strategies?

A cost-effectiveness analysis compares both costs and outcomes of two or more strategies. Outcomes are expressed unvalued in clinical units of effect such as number of decays prevented when evaluating a fissure sealing program. A cost-utility analysis is a special form of cost-effectiveness analysis. In a costutility analysis outcomes are expressed in constructed units of effect such as quality-adjusted tooth years (QATYs) (BIRCH 1986). A weighting factor (utility) between zero (missing tooth) and one (sound tooth) is used for quality-adjustment. For example, if patients choose tooth-colored composite resin instead of an amalgam for the restoration of a cavity in a premolar, the patients' preferences could be measured applying an appropriate method as described elsewhere (SENDI et al. 1997). Let us assume a hypothetical utility of 0.9 for a tooth restored with composite resin and a utility of 0.7 for a tooth restored with amalgam. An amalgam filled tooth functioning ten years without any further problems would represent 7 QATYs (0.7×10 years). But a tooth with a white filling would represent 9 QATYs after ten years of problem-free use (0.9×10 years). This simple example shows how measures of quantity and quality can be combined and expressed as a constructed unit of effect. This enables comparability across different interventions. It is most useful when alternative treatment strategies produce different types of outcomes.

It is emphasized that a cost-effectiveness or cost-utility analysis always means an explicit comparison of treatment alternatives in terms of both costs and outcomes. A new therapy could be compared with the standard treatment strategy or with the no intervention strategy. A more universal outcome measure than QATY would be required for addressing complex problems in implant and maxillofacial prosthodontics, orthodontics or oral surgery. Ideally, an instrument that could be used to compare any aspect in modern dentistry, including esthetics, would be preferred, making it easier to compare outcomes across dental specialties. However, such an outcome measure still remains to be developed. An alternative approach would be to express health-related outcomes in terms of willingness-to-pay (GAFNI 1991). The first study applying these methods in dentistry has recently been conducted by MATTHEWS et al. (1999). However, valuing health-related outcomes in monetary terms is crucial for conducting a cost-benefit analysis, but not a cost-effectiveness analysis which is the focus of this article.

The viewpoint or perspective of the analysis determines the type of costs that should be considered when performing a cost-

effectiveness analysis (DRUMMOND et al. 1997b). A cost-effectiveness analysis may be conducted from any of the following perspectives: the society at large, a third-party payer (e.g. an insurance company), the dental community (providers of dental care), a dental company, a managed care group or a patient population. Of course, some perspectives may conflict with others. For example, if health insurance companies would reimburse patients for treatment costs in fixed prosthodontics but not for costs arising from yearly visits to the dental hygienist, then this reimbursement policy would lead to an increased demand in expensive treatment modalities at the expense of a low demand in preventive services. Easy access to expensive treatment alternatives at a moderate price is desirable from the patient's point of view, but obviously leads to higher insurance costs and overall medical costs, which is undesirable from the third-party payer's and societal perspective. Generally, the societal perspective is considered the most appropriate viewpoint to inform decision and policy makers (DRUMMOND et al. 1997b). A cost-effectiveness analysis from a societal perspective aims to include all types of costs. Economic consequences of choosing an alternative may include savings as well as direct medical costs (e.g. costs for a surgical intervention), direct non-medical costs (e.g. transportation costs), indirect costs (e.g. lost work productivity) and intangible costs (e.g. pain and suffering).

Although Maryniuk et al. (1988) call their study a cost-effectiveness analysis, it is a cost analysis by definition (Drummond et al. 1997b). A cost-effectiveness analysis compares both costs and clinical outcomes of two or more strategies. But in the presented analysis a proper outcome measure such as QATY does not exist. The authors modeled the costs over a time horizon of 42 years. The average yearly costs over a patient's lifetime, assuming a life-expectancy of 42 years, is calculated and reported as 42-year utility. The perspective of the analysis has not been explicitly stated, but we assume the patient's perspective because only direct medical costs to the patient were included.

2. Were the costs and outcomes properly measured and valued?

A cost-effectiveness analysis is often based on outcome data reported in clinical trials or meta-analyses (PALMER & SENDI 1999). Randomized controlled trials represent the gold standard for evaluating the efficacy of a treatment. But the external validity (i.e. the relevance of the results to the general population) of a randomized controlled trial might be limited (DRUMMOND 1998). When performing an economic evaluation from the societal perspective, it is often more important to adhere to the external validity of the selected studies which document the effectiveness of the treatment under a real-world setting (DRUM-MOND 1998). For example, a structured oral hygiene motivation program will likely be much more successful in clinical trials under controlled circumstances than in general practice. A study with a high degree of external validity, on the other hand, improves the likelihood that the results are applicable to general practice.

The number of economic evaluations conducted alongside clinical trials is increasing. However, these trials might not be the best place to collect cost data and have only a limited follow-up period (Coyle et al. 1998, Ellwein & Drummond 1996). Economic evaluations based on mathematical modeling are therefore often needed to extrapolate beyond the endpoint of clinical trials and to adjust for the desired degree of external validity (Buxton et al. 1997). A mathematical model should be validated before it is used for policy recommendations (Sendi et al.

1999a). In addition, the validity of a cost-effectiveness analysis might be questionable when outcomes of a strategy are modeled over a period that is by far beyond the follow-up period of the original clinical trial. The time horizon used depends on the therapy under evaluation. For example, the effect of water fluoridation can accrue over a lifetime, but the relevant effects of preoperative anesthesia are much shorter in comparison, and an extrapolation of the results over a patient's lifetime may be inappropriate.

Physical quantities of resources consumed by different treatment strategies should be reported separately from their unit prices. This facilitates a proper interpretation of the results. Resource consumption and unit costs often widely differ by geographical area and make the generalizability of the results of a cost-effectiveness analysis difficult. All costs considered in the analysis should be listed in tables or mentioned in the text. The viewpoint of the analysis determines the type of costs relevant for the analysis (DRUMMOND et al. 1997b). Not only costs incurred to provide the therapy, but also future costs associated with the therapy are helpful to derive an unbiased estimate of resource consumption.

All future costs and outcomes should be discounted (DRUM-MOND et al. 1997b). A specified amount of money or health benefit in the future is less worth than today. The US Panel recommends an annual discount rate of 3% (Weinstein et al. 1996). However, other health economists emphasize that the discount rate depends on many factors, including the perspective of the analysis (Krahn & Gafni 1993).

The probabilities used in the analysis are all based on expert panel estimates. This is the weakest form of evidence (Sackett et al. 1997). The authors do not report the unit prices for the restoration procedures used. The modeling assumptions and limitations have been made explicit. Because the analysis by Maryniuk et al. (1988) is not a cost-effectiveness analysis, only expected lifetime costs can be compared. Costs were discounted at an annual rate of 5%.

3. Was appropriate allowance made for uncertainty in the analysis?

In a sensitivity analysis input parameters are varied over a defined range. This allows us to test how sensitive the model is to key assumptions and data variability. In the above-mentioned example, we might not be sure about the exact utility (0.9) of composite resin. We could vary the utility in a range between say 0.8-1.0 to see how the incremental cost-effectiveness ratio changes with changes in utility estimates for composite resin. The ranges for sensitivity analysis should be justified. A model is said to be robust when various values for input parameters do not have a major impact on the results and conclusions of the analysis. This form of sensitivity analysis, although not without limitations, is predominant in published cost-effectiveness analyses. Recently, researchers have begun to develop and apply more sophisticated statistical methods such as bootstrapping (SENDI et al. 1999b) or Bayesian methods of analysis (CRAIG & NEWTON 1997) to assess overall parameter uncertainty. Bayesian analysis relies on the idea that uncertainty can be described by a distribution. Bootstrapping is a computer-intensive resampling technique and has become popular with the advent of cheap computational power.

Univariate sensitivity analysis has been performed on the probability of amalgam replacement, the probability of endodontic therapy, and the discount rate. No sensitivity analysis has been conducted on treatment costs. The authors vary the probabilities over a very large range. But it is unclear whether a sensitivity analysis on multiple parameters at once (multivariate) was conducted. A sensitivity analysis on more than three parameters becomes difficult to interpret. Bayesian methods of analysis would have been useful to assess overall parameter uncertainty. However, this may reflect an assessment of the uncertainty of expert opinions rather than an assessment of uncertainty of the unknown true probabilities.

4. Are estimates of costs and outcomes related to the baseline risk in the treatment population?

The baseline risk in the treatment population often dramatically influences the costs and outcomes in a cost-effectiveness analysis. For example, heavy smokers are likely to have a higher failure rate after implant insertion than non-smokers because of their reduced wound healing capabilities and higher risk of infection. This would translate into a higher cost-effectiveness ratio for this treatment modality in this patient-subgroup compared to non-smokers. The cost-effectiveness ratio would increase and become less attractive in this case.

Factors influencing the durability of a restoration such as oral hygiene were not explicitly modeled. We do not further evaluate the study by Maryniuk et al. (1988) using the items 5–7 since the analysis is not a formal cost-effectiveness analysis.

5. What are the incremental costs and outcomes of each strategy?

In order to compare costs and outcome of two or more strategies, it is essential to compute the incremental cost and incremental outcome which is the difference in costs and outcomes observed between two strategies. Thereafter, the results may be located on a 3×3 matrix with nine cells to categorize the study (Fig. 1). A new strategy could be more, equally or less expensive and more, equally, or less effective than a control strategy. A strategy that falls into category 1 is cheaper and more effective and is said to be strongly *dominant*. An alternative therapy that falls into category 2 is more expensive and less effective than a control therapy. Such a therapy is said to be strongly dominated. The categories 3–6 represent similar situations where a therapy is dominant or dominated compared to a control strategy. A therapy falling into category 1-6 requires no further analysis. However, if results fall into the cells 7, 8 or 9, the incremental cost-effectiveness ratio yields more insight. The typical situation is a therapy that falls into category 7. A new treatment policy is often both, more expensive and effective than the control therapy. The incremental cost-effectiveness ratio should be distinguished from the average cost-effectiveness ratio. In contrast to the incremental cost-effectiveness ratio, the average cost-effectiveness ratio does not allow a proper comparison of treatment alternatives. The incremental cost-effectiveness ratio implies a comparison of two strategies in terms of both costs and clinical outcomes. The average cost-effectiveness ratio, on the other hand, does not imply a comparison of alternatives and may lead to a completely different ranking of strategies as shown by Detsky & Naglie (1990).

The incremental cost-effectiveness ratio should inform decision makers about the extra benefit that could be bought at any extra cost. However, there is an ongoing debate about the correct interpretation of incremental cost-effectiveness ratios (BIRCH & Gafni 1993). The incremental cost-effectiveness ratio is calculated by dividing the incremental costs by the incremental effectiveness. For example, let us assume that patients prefer tooth-

Fig. 1 Possible outcomes when comparing two treatment options in terms of both costs and effectiveness

		Incremental effectiveness				
		higher	same	lower		
Incremental cost	higher	7	4	2		
	same	3	9	5		
	lower	1	6	8		

Cells 1 and 2 indicate strong dominance of a strategy (cell 1 = accept strategy, cell 2 = reject strategy). Cells 3, 4, 5 and 6 indicate weak dominance of a strategy (cells 3 and 6 = accept strategy, cells 4 and 5 = reject strategy). Cells 7, 8 and 9 indicate no dominance of a strategy (cell 7 = is the higher effectiveness worth its cost? cell 8 = is the lower effectiveness acceptable at the lower cost? cell 9 = neutral on effectiveness and cost. Are there other reasons to adopt the new strategy?)

colored composite resin (utility 0.9) over amalgam (utility 0.7) for the restoration of a premolar. And let us assume that a composite resin would last for ten years and an amalgam for eleven years. An amalgam would cost 200 CHF and a composite resin 300 CHF. The incremental effectiveness of composite resin over amalgam would be 1.3 QATYs (0.9×10 years–0.7×11 years) and the incremental costs 100 CHF (300 CHF–200 CHF) respectively. Hence, the incremental cost-effectiveness ratio in this hypothetical example is 76,9 CHF per QATY (100 CHF/1,3 QATYs) (Table II). If amalgam would be the standard therapy, changing from amalgam to composite resin as the new therapy would cost 76,9 CHF per QATY gained. On average, the patient would pay an additional 77 CHF for the extra benefit of one QATY when choosing composite resin instead of amalgam.

6. Do incremental costs and outcomes differ between subgroups?

The cost-effectiveness of a therapy depends on whom it is provided to (see question 4). The baseline risk of morbidity may vary from one patient subgroup to another. In consequence, the cost-effectiveness of a therapy often simultaneously changes from one patient subgroup to another. This variation among patient-subgroups might influence the decision to whom priority should be given for certain treatment modalities.

7. How much does allowance for uncertainty change the results?

The 95% confidence interval is often used as a range for sensitivity analysis when data from clinical trials are used. Estimates based on assumptions or expert opinion should be evaluated

Table II Results of a hypothetical cost-effectiveness analysis comparing amalgam versus composite resin

Strategy	Costs ¹	Effectiveness ²	Incremental Costs	Incremental Effectiveness	Incremental Cost- Effectiveness Ratio
Amalgam	200 CHF	7.7 QATYs			
Composite Resin	300 CHF	9 QATYs	100 CHF	1.3 QATYs	76.9 CHF/QATY

Costs in Swiss Francs (CHF)

over a wide range of values. Caution should be used in drawing conclusions from a model tested with unjustified narrow parameter ranges used in the sensitivity analysis.

Comments

The presented study by Maryniuk et al. (1988) compares the estimated lifetime costs for two strategies and is therefore a cost analysis (Drummond et al. 1997b). The authors fairly state the assumptions and limitations of the study. Since all estimates are based on expert opinion and not on clinical trials, the main conclusion is that better data are needed for better clinical decision making. Univariate sensitivity analysis has its limitations (Felli & Hazen 1998) and conclusions from such a model should be interpreted with caution, in the absence of high quality probability data. At the time this analysis was published, however, these techniques were not as advanced and popular as they are today. The development of new methodological instruments in dentistry is an important task for the future.

Zusammenfassung

Immer mehr wissenschaftliche Publikationen beleuchten die ökonomischen Aspekte von Medizintechnologien. Die Kosteneffektivitäts-Analyse ist ein hilfreiches Mittel, um die klinische Entscheidungsfindung unter Berücksichtigung ökonomischer Überlegungen zu optimieren. Evidence-Based Medicine, ein zurzeit populäres Konzept, beruht auf der kritischen Beurteilung von Studien und deren Anwendung in der Praxis. In der vorliegenden Arbeit wird eine ökonomische Evaluation im zahnärztlichen Bereich von MARYNIUK et al. (1988) kritisch beurteilt (Replacement of amalgams with crowns: a cost-effectiveness analysis), wobei gleichzeitig die hierzu notwendigen theoretischen Grundlagen vermittelt werden. Um zu eruieren, ob die Resultate einer Kosteneffektivitäts-Analyse stichhaltig sind, sind folgende Fragen zu stellen: 1. Wurde eine Analyse der zu vergleichenden Strategien durchgeführt, die alle Kosten und relevanten klinischen Endpunkte berücksichtigt? 2. Wurden die Kosten und klinischen Endpunkte korrekt gemessen und bewertet? 3. Wurde eine ausgedehnte Sensitivitätsanalyse durchgeführt? 4. Steht die Schätzung der Kosten und der klinischen Endpunkte in Beziehung zum basalen Erkrankungsrisiko der behandelten Population? 5. Was sind die inkrementellen Kosten und die inkrementelle Effektivität der Strategien? 6. Unterscheiden sich die inkrementellen Kosten und die inkrementelle Effektivität nach Subpopulationen? 7. Wie stark ändern sich die Resultate in einer Sensitivitätsanalyse?

Résumé

Le nombre des évaluations économiques trouvées dans la littérature médicale et dentaire est en augmentation. Les analyses

² Effectiveness in quality-adjusted tooth years (QATYs)

coût/efficacité sont utiles pour renseigner les preneurs de décision au sujet de la politique de la santé. Plusieurs principes d'analyse coût/efficacité publiés pour le domaine dentaire sont passés en revue. Une meilleure compréhension des rouages de ces principes et des étapes impliquées dans l'analyse critique des études économiques concernant la santé devrait faciliter la prise de décision au sein de la communauté dentaire.

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