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Unlocking Efficiency: The Art of Cost Effectiveness: A Narrative Review

[1] Dr. Soundarya Prabhakar, [2] Dr. Thiruppathy Manigandan

[1] PhD Scholar, Bharath Institute of Higher Education and Research, Tamil Nadu, India
[1] Assistant Professor, Department of Public Health Dentistry, Tagore Dental College and Hospital, Tamil Nadu, India
[2] Professor, Department of Oral Medicine and Radiology, Sree Balaji Dental College and Hospital, Tamil Nadu, India

Corresponding Author: **Dr. Soundarya Prabhakar**

Abstract

In addition to being widely used, cost-effectiveness analyses are intuitive tasks that people perform on a daily basis. Health economic assessment is becoming more and more crucial in normal medical care because of population growth, growing demands, progressively decreasing resources, and ongoing therapeutic advancements. In order to ensure an efficient evaluation of innovative interventions for public health policy, the health economic assessment needs to analyse the results and costs of actions and technology with as much objectivity as feasible. Put differently, it is imperative to ascertain the extent to which patients or society are prepared to pay for innovative interventions in relation to current options, considering the available means. Furthermore, under fixed budget health care systems, rising costs may drive out other currently offered health care services. Researchers must employ conventional procedures and interpretations in consideration of regional characteristics based on social and economic determinants as well as clinical practice in order to undertake such assessments. Using such a strategy could be crucial to converting the existing healthcare system to a value-based one. Concepts regarding the significance and methods of health economic evaluation in clinical practice will be covered in this narrative review.

Keywords: Cost Effectiveness, Health Economics, Budget, Health Care, Analysis

1. Introduction

Disease treatment and prevention necessitate ongoing advancement, which is often founded on new ideas and technological advancements. Even in industrialized nations, there are significant access and cost issues when deciding whether new or existing interventions or programs to allocate funds to. These issues impact patients, legislators, payers, and doctors [1].

In this context, cost-effectiveness analysis emerges as a valuable technical instrument for decision-making, benefiting physicians in their clinical practice as well as the decision-making authorities of health care insurers. Contrary to popular belief, the question is not how much to charge for the value of a life, but rather how much society and patients are willing or able to pay for novel interventions in comparison to current treatments, or even whether patients and health care systems can save money by using them. To avoid a "state of crisis" that has been documented in expensive health care systems, better financial resource allocation may be necessary for both individual patients and the general public. This could even lead to a decrease in the number of deaths [2, 3].

In situations where objective and technical resources are lacking to support their decision-making, physicians may encounter a predicament known as the "Sophia choice" in certain nations [4]. Beyond them, assessing efficacy in clinical practice without taking into account the trade-off between lower incident rates and higher expenses might not be enough to support the introduction of novel therapies [5, 6]. Better results are not always the consequence of increased spending, either. Comparing the United States to other high-income nations, for example, reveals that, despite significantly higher health spending, the country has a lower health-adjusted life expectancy—that is, the number of years in full health that a person can expect to live given the current conditions of morbidity and mortality [7].

Even though the concept of cost-effectiveness in non-medical issues was first introduced by the US War Department in 1886 [8] and cost-effectiveness research in Western medicine began in the 1930s [9], the application of incremental cost-effectiveness ratios (ICER) in the decision-making process has not reached a consensus due to regional differences in cost-effectiveness thresholds [5, 10, 11].

2. Health Economic Evaluation

There are two methods to estimate the economic health assessment, depending on the cost and efficacy statistics that are available. One possibility is that it draws from real (clinical) data, such as those from clinical trials or observational studies. However, it might be based on computational modelling, which would include information from a variety of sources, including expert opinions, systematic reviews, epidemiological studies, and real (clinical) data. Such assessments frequently include the combination of data from several sources. Decision models show how patients move through various stages of health and provide a schematic picture of the complexity of the real world [4, 5, 12].

The piggyback study, which is an economic evaluation study combined with a clinical trial, has benefits and drawbacks [13]. Randomization and blinding are therefore usually advantageous to them, and it could be less expensive and simpler to incorporate an economic component into a prospective clinical research than it is to fund an economic review outside of a trial. The primary drawbacks, however, are that clinical trials typically do not accurately represent "real-world" practice, and the time horizon is constrained to the study's follow-up period. In these situations, computer models might be applied supplementary to different patient populations with the same ailment or to assess long-term impacts.

The modelling strategy has the drawback of being predicated on assumptions that cannot be verified during the trial, even though it is frequently the only method available to address the a forementioned inadequacies of clinical trials [14, 15]. Static models, like decision trees and Markov modelling, as well as dynamic models and microsimulation models, are the primary modelling techniques that are utilized [14-16]. Naturally, it is vital to assess the suitability and quality of these models in terms of the data that was available for usage, the validity of the models in comparison to the outcomes of other studies or models, the transparency of the descriptions provided, and the rationale behind the selection of particular models [16].

However, since the field of health is always evolving, modelling methods predicated on present hypotheses might not hold true in the future. New items or technology can therefore provide new outcomes and change the entire modelling scenario. However, a detailed discussion of these modelling methodologies is outside the purview of this paper and can be found elsewhere [16].

2.1 Types of Methods of Economic Evaluation

The four general areas of economic assessment analyses are cost-minimization, cost-effectiveness (CEA), cost-utility, and cost-benefit analysis [4].

2.2 Cost

Finding, measuring, and appraising the many kinds of resources utilized is how costs are determined. There are several categories of expenses: Direct medical costs, non-medical direct costs, indirect costs, and intangible costs [10]. Two methodologies—micro-accounting, macro-accounting, or a combination of both—are used to determine direct medical costs. Through the use of a methodology known as micro-costing, each resource used is calculated and given a unit cost. This process produces cost estimates that are more detailed and allow for the identification of the inputs used by focusing on the specific patient [17, 18].

On the other hand, national registries like Hospital Information Systems and the Ministry of Health's Outpatient Information System use macro-costing, which identifies the most pertinent resources at a high level of aggregation and provides an average of treatment costs for each category of disease. The benefit is that, in most cases, it is a more practical approach than micro-costing [19, 20]. It may, however, be less sensitive to slight variations in direct costs due to its lower degree of cost estimate accuracy [17, 18]. However, in order to make micro-costing more feasible, it may exclude those expenditures that are thought to be unaffected by the therapy or contribute very little overall.

There are two more categories for the methods: Top-down and bottom-up. The System of Management of the Table of Procedures, Medications, Orthotics and Prostheses, and Special Materials of the Unified Health System or Medicare's Bundled are only a few examples of the comprehensive sources from which resources are valued in the top-down estimating process. As an alternative, healthcare services, labour, or purchase contracts are used to value human, material, and financial resources in the bottom-up method [17].

2.3 Adjustments of Cost Data

Adjustments should be made for costs and advantages that occur at different times, in different countries, and in different currencies. Additionally, inflation may have an impact on costs collected throughout different time periods. It might be required to adjust the resources or value them all using the same base year, which is typically the present, in order to eliminate the effects of inflation from the analysis. The consumer price index or gross domestic price deflators might be used for this modification [10, 21].

Additionally, the value of future benefits and expenditures at various time points must be converted via discounting. When using projection in the project, as in a Markov model, adjustments must be made for this. The literature continues to discuss the discount rate, which varies depending on the country [22].

2.4 Converting Cost into a Common Currency

Furthermore, the disparities in price levels among nations are not reflected by a straightforward conversion of all national unit costs using market exchange rates from currency markets into a single common currency. Economists frequently choose to use "international dollars," a fictitious currency, while doing so [23].

According to the theory, a specific amount of foreign currency should be able to purchase approximately the same quantity and calibre of products and services in every nation [5, 21, 24]. "Currency conversion rates known as purchasing power parities are used to remove country-specific price differentials and equalize the purchasing power of various currencies [24].

2.5 Effectiveness: Calculating Quality Adjusted Life Years (QALY)

"Manually obtaining QALY can be accomplished by multiplying the average utility of each value between two consecutive time measurements by the time interval between the measurements, then adding up all the values [25, 26]." If every patient has an equal chance of finishing each period, the average QALY of the group can be computed for each

time period or the total of all the periods combined. It is difficult to estimate the mean QALY since some people have their survival time suppressed.

As a result, such censored observations will prove to be useful when estimating QALY, rendering the standard Kaplan–Meier calculation insufficient. One option in this situation is to think about the other approach [26]. A technique to calculate the mean QALY was created by the same authors [28]. Therefore, variations in the corresponding means can be used to analyse differences in QALY between groups. The area under the curve should be calculated using the time intervals between the collection of baseline utility data and the patient's death. Patients with baseline utility data who passed away prior to the first follow-up utility assessment ought to be included. It can be required to balance QALY [29, 30].

2.6 Sensitivity Analysis

Sensitivity analysis is an effective way for analysing the effects of data uncertainty resulting from various study approaches, data sources, and data deficiencies or excesses, among other factors [5, 19]. By adjusting several factors, it seeks to identify how robust the results are and how much a variation can affect the outcome. Sensitivity analysis also makes it possible to investigate how results can be generalized [31]. Data variations should be supported by a review of the literature, advice from experts, or the use of confidence intervals [5, 19, 31]. Both probabilistic and deterministic sensitivity studies are possible [5, 32, 33].

More complex and strongly advised probabilistic sensitivity analysis (PSA) uses the Markov Chain, Monte Carlo simulation, or bootstrapping to simulate [34]. PSA can be carried out in a decision-analytic model or in an economic evaluation in conjunction with a clinical study. Because PSA allows for the simultaneous assessment of the joint uncertainty across all model parameters, it has become the industry standard. Our main focus, the bootstrap approach, will be covered in full below.

2.6.1 Bootstrap Method

It is inappropriate to generate confidence intervals using ordinary statistical procedures because incremental ICER and incremental cost-utility ratio (ICUR) are fractions and the underlying variables are typically not normally distributed. Examining alternative methods, such as nonparametric bootstrapping, is one potential remedy [35].

A statistical strategy known as the bootstrap method involves drawing equal-sized random samples to serve as the initial sample. While some initial observations may appear more than once in a bootstrap sample, some original observations might not be selected. While the number of samples is not specified, it is advised to have at least 1000 [36]. The bootstrap method's fundamental idea is to handle the study sample as though it were a conceptual population. Drawing conclusions from these samples is preferable to speculatively assuming anything about the underlying population. Understanding the distribution of findings and the estimation's accuracy is possible via bootstrapping.

An array of techniques, including the normal approximation approach, percentile method, bias-corrected percentile method, and expedited method of bias correction, were developed for the purpose of creating boot-strap confidence intervals. But which of those approaches is best depends on the particular use case. Numerous writers offer a thorough explanation of every method in addition to a synopsis of its benefits and drawbacks [37]. It is outside the purview of this essay to discuss each of these strategies in detail.

Using asymptotic results (i.e., large sample results) to draw inferences does not need the application of distributional assumptions for the data, which is one of the key benefits of the bootstrap approach. Actually, any estimator can be used with this method, including complex ones like ICER or ICUR. When constructing confidence intervals using bootstrap, there are instances where the necessary confidence level is not achieved, necessitating the employment of more advanced techniques. Additionally, the approach might not work with tiny sample sizes. Since cost and outcome statistics are frequently not normally distributed, media values are frequently used because they are heavily impacted by extreme values [21].

When a large number of samplings is taken, as is advised, averages are (almost) identical to the outcomes of parametric tests. For this reason, the main purpose of bootstrap is to provide insight into the distribution of data and the probability of crossing specific thresholds.

2.7 Incremental Cost-Effectiveness Planes

With the use of a scatter plot, cost-effectiveness analysis with the nonparametric bootstrap technique can be visually depicted as incremental cost-effectiveness planes. Plotting the incremental costs and incremental effectiveness of an alternative therapy in relation to the comparator and indicating the degree of ambiguity surrounding the alternative therapy's cost-effectiveness are done using incremental cost-effectiveness planes. On the x-axis, the incremental effectiveness is displayed, and on the y-axis, the incremental cost. Every bootstrapping sample corresponds to a single point on the scatterplot in the four quadrants that are explained below.

2.8 Limitations of CEA

Some authors contend that a circumscribed ICER threshold ignores the possibility that decision-makers may opt to apply a different ICER limit based on the nature of the illness, the type of drug, and the setting of the decision-making process [34, 35]. Actually, some nations use the variable threshold ICER model. It is thought that because medications have a larger societal value or because they treat rare disorders for which there is no other treatment, the ICER threshold should be higher. Furthermore, ICER thresholds are set arbitrarily [35].

Additionally, a highly selected group is frequently included in clinical studies for a set amount of time, completely ignoring a suitable lifetime horizon. As such, the data might not accurately represent all patients in a real-world scenario who have a certain ailment, or they might be deemed insufficient to assess occurrences [10]. Furthermore, projections typically presuppose that the dosages, surrogate measures, and patient characteristics are uniformly distributed throughout time, while this is frequently not the case. Moreover, missing data are common. Although they might not fully account for the clinical complexity, assumptions might be required. Furthermore, projecting data can introduce bias, and long-term estimates require more data to support them—data that may not always be accessible, especially for novel medications.

3. Conclusion

Decision-making in healthcare and probably in the future in clinical practice will depend more and more on CEA. Because resources are few, efforts should be taken to ensure that health care practitioners take CEA into account while making every day clinical decisions. This would not only be beneficial for the creation of public policies. In clinical practice, shared decision-making should be promoted if patients are fully informed on the effectiveness and cost-effectiveness of all available treatment options, especially if they entail out-of-pocket costs.

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