CHAPTER 1

Principles and methods

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PERSPECTIVE, COSTS, OUTCOMES AND DISCOUNTING IN PHARMACOECONOMIC EVALUATIONS

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Aspects of the methodology and controversies regarding economic evaluations in the field of health care, focusing on cost-utility analysis, are highlighted. In particular the topics of perspective, costs, relevant outcomes and discounting are addressed.

Contrary to what many people think, economics is not about money. Economics is the social science concerned with the efficiency and equity consequences of alternative allocations of scarce resources. The general idea in economics is that our limited resources should be used optimally, and that they are used optimally when (social) welfare, utility, is maximised. In economics the term 'utility' is seen as the ultimate good that individuals aim to obtain in life, and has sometimes been equated with happiness or (preference) satisfaction [1]. When a 'change' increases social welfare it is deemed efficient, if not, it is labelled inefficient and preferably should not take place. A practical way of deciding whether or not social welfare is increased due to the 'change', is by considering whether the benefits outweigh the costs of that change when they are both expressed in a comparable way, often in terms of money. While this practical test requires some bold assumptions, e.g. that all involved individuals attach the same utility value to money, this is the basis of the traditional type of economic evaluation (cost–benefit analysis). The question such an analysis seeks to answer is whether social welfare will increase when a 'change' is implemented. This is assumed to be the case when the monetary value of the benefits exceeds the monetary value of the costs.

Normally, in health care, economic evaluations take the form of a cost-effectiveness analysis (CEA) or cost-utility analysis (CUA). Then, the benefits are not expressed in monetary terms, but rather in natural units, e.g. percentage blood pressure reduced or quality-adjusted life years (QALYs). While this has the advantage of avoiding putting a 'price' on health and life, which probably explains the popularity of these types of economic evaluations in health care, it also comes at a price, i.e. costs and benefits are no longer directly comparable. Therefore, it is no longer possible to directly determine whether funding some new intervention, e.g. new drug, will increase social welfare, as in a cost–benefit analysis. Now, this remains implicit and depends on the value placed on QALYs or other effects, often referred to as the threshold or the lambda. By comparing the incremental costs and benefits of an intervention compared to a relevant alternative, we only provide the decision maker with a ratio of costs and effects and leave it up to this decision maker to decide whether this ratio implies welfare improvement or not.

PERSPECTIVE

Looking at the theoretical roots of economic evaluations, it is not surprising that most textbooks recommending economic evaluations adopt a societal perspective. This perspective implies that all relevant costs and outcomes need to be considered in the analysis. The reason for this is clear. Only if we consider all relevant costs and effects in the analysis can we be sure that our conclusions regarding the change in social welfare are indeed correct. Leaving out such changes makes such a final judgment impossible. It is therefore important to consider the dominant definition of the societal perspective in health economic evaluations: "*When a CEA is conducted from the societal perspective, the analyst considers everyone affected by the intervention and counts all significant health outcomes and costs that flow from it, regardless of who experiences the outcomes or costs*" [2]. Therefore, the outcomes of interest are seemingly narrowed down to health only.

While the societal perspective is advocated frequently, it is not adopted by all economic evalua-

Economic evaluations seek to inform decision makers about the welfare impact of a medical intervention.

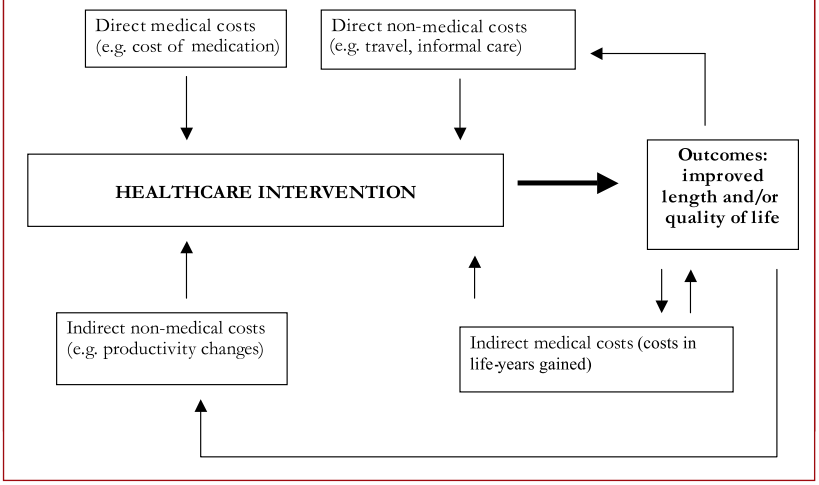
tions. Sometimes simply because of poor practice, such as leaving out relevant costs or outcomes without justification, but often because another perspective is deliberately chosen. Normally, this will be the healthcare perspective. In some jurisdictions, such as England, this perspective is advocated by the relevant authorities, i.e. the National Institute for Health and Clinical Excellence. In this case the analysis will typically include only those costs falling under the healthcare budget and leave out others. The rationale behind adopting such a narrow perspective is to try to inform a healthcare decision maker about the optimal allocation of their budget. Whether or not this implies that such decision makers would want to remain ignorant regarding other societal costs and benefits related to their actions remains a matter of debate [3].

Costs

Costing normally entails three distinct phases:

- The identification of relevant costs, involving recognition of those categories and items that are important for a particular study.
- The accurate measurement of the units of resources used, i.e. time of formal or informal caregivers, days absent from work, number of hospital visits, etc. This is often not straightforward and is a crucial part of an economic evaluation.
- The valuation of these units. The costs per resource unit need to be determined. This may be difficult for some types of costs particularly since prices may not exist, e.g. informal care, or may not reflect real economic costs, e.g. hospitals tariffs. The degree of effort spent on finding appropriate values depends on the importance of a specific cost category [4].

Figure 1: Different costs in economic evaluations



In economic evaluations, different types of costs are normally distinguished (see Figure 1). However, different categories of costs have been proposed, none of which are perfect, so a common approach is used here.

The *direct medical costs* are costs directly related to the intervention under study falling within the healthcare sector and include formal caregivers' time, diagnostic tests, drugs and other hospital materials, independent of who is financing these costs. This cost-category is a central element in any economic evaluation and relevant when adopting a societal or a healthcare perspective.

The *direct non-medical costs* are also directly related to the intervention under study, but fall outside the formal healthcare sector. Examples are patients' travel costs, costs of home adaptations and informal care. While these costs are relevant when adopting a societal perspective, they may be considered irrelevant when adopting a healthcare perspective.

The category *indirect non-medical costs* mainly refers to so-called productivity costs, which occur when an illness or treatment leads to absenteeism, presenteeism (reduced productivity at work), disability or premature death in productive persons, paid or unpaid. These costs (or savings!) can be substantial, but there is much debate regarding how to measure and value them [5]. The inclusion of these costs is relevant from a societal perspective, but not from a healthcare perspective.

Finally, *indirect medical costs* comprise medical costs that result from care consumption in gained life years. When an intervention prolongs life, medical costs are normally incurred during these years. These costs may either be related to the intervention under study, e.g. costs of drugs to avoid rejection of the new heart after a heart transplantation; or unrelated, e.g. costs related to a hip fracture after successful bypass surgery. While these costs are relevant from a societal as well as from a healthcare perspective, only the inclusion of related indirect medical costs is undisputed. Whether unrelated costs should also be considered remains a matter of much debate [6] and the measurement and valuation of these costs is not straightforward.

Outcomes

When presenting the decision rule under CUA, the outcome of interest in an economic evaluation

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is normally assumed to be a patient's health gain, measured as QALY gains. However, one may consider such a focus to be too narrow. Indeed, the improvement of a patient's health will be the central aim in most healthcare interventions. Still, other outcomes may matter as well. A first logical extension of the outcomes considered in an economic evaluation may be the health of others. The US guidelines already encourage analysts "*to think broadly about the people affected by the intervention and begin to include health-related quality of life effects of significant others in sensitivity analyses when they are important*" [2]. Two logical groups of significant others, for which the interest is growing, are informal caregivers and family members. Both may experience health changes due to the mere fact that someone in their social environment is ill (family effect) or due to burdensome care giving tasks (caregiver effect) [7]. It is important to consider such effects and to avoid treating patients as isolated individuals [8] both from a societal and from a healthcare perspective.

Discounting

Often, an intervention will not only cause current costs and effects but also future ones. In economic evaluations such future costs and health gains are commonly weighted in relation to the time at which they occur. This procedure is called 'discounting' and is a reflection that current costs and effects are more important than future ones. A present value of a stream future costs, or effects is calculated like this:

$$V = \sum_{t=0}^{T} \frac{Ct}{(1+r)^t}$$

,where Ct is the costs in year t and r is the discount rate on costs. Normal discount rates for costs, and effects, are in the range of 3–5%. The influence of discounting on final cost-effectiveness may be substantial, especially when effects occur far in the future while costs are current, e.g. in the case of vaccinations [11]. Although the exact height of the discount rates (the rate of depreciation of future costs and effects) differs between different national guidelines, all prescribe discounting. Currently, there is much debate about the question of whether costs and effects should be discounted with the same discount rate. While it is still standard practice to do so, the rationale for this practice is increasingly questioned. Given that the value of health (QALYs) is likely to increase over time, which is not reflected in CUAs, some have argued that future health should be discounted at a lower rate than costs which can have a profound impact on cost-effectiveness results [12].

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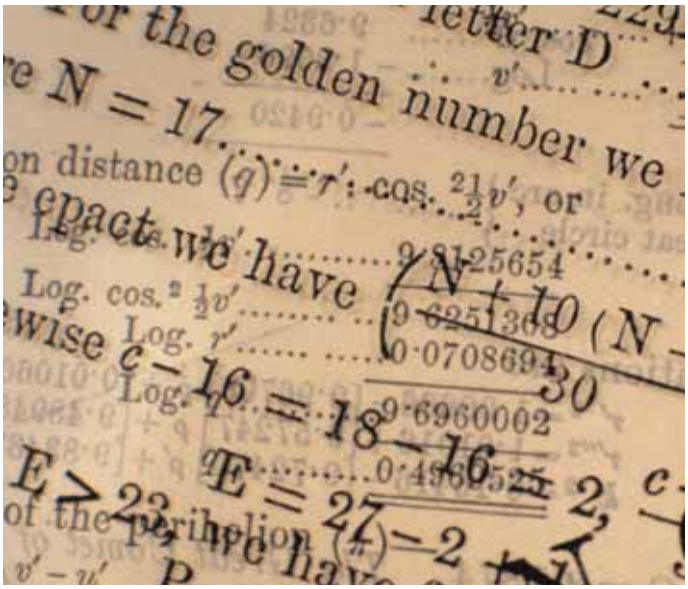
Other relevant outcomes may relate to well-being effects in patients and others. For instance, patients may prefer one of two equally effective medications if one has to be taken three times a day while the other needs to be taken just one time per week. Patients attach value to such characteristics, which should not be ignored, at least not in an analysis adopting a societal perspective. In a healthcare perspective, the relevant outcomes are sometimes explicitly narrowed down to health gains, which would deem any broader well-being changes irrelevant. It is important to note that the significant others may also experience well-being changes which may be important as well when adopting a societal perspective [9].

The monetary value of the relevant outcomes, including the QALY, also needs to be considered in this context. In the end a decision maker needs to decide whether social welfare is improved by an intervention on the basis of the incomplete information provided by the economic evaluation. Without knowledge regarding the value of the QALY which is likely to be context specific, such decisions may be deemed impossible. So far, however, we know fairly little about the value of health and other care-related outcomes [10].



Conclusion

Economic evaluations seek to inform decision makers about the welfare impact of a medical intervention. Given the underlying aim it is therefore important that they are well-designed, and both consider and weigh all relevant costs and effects appropriately.

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