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Appraisal of health care: from patient value to societal benefit

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Abstract

Aim This paper summarizes the deficiencies and weaknesses of the most frequently used methods for the allocation of health-care resources. New, more transparent and practical methods for optimizing the allocation of these resources are proposed.

Method The examples of quality-adjusted life years (QALYs) and efficiency frontier (EF) are analyzed to describe weaknesses and problems in decisions regulating health-care provision. After conducting a literature search and discussions with an international group of professionals, three groups of professionals were formed to discuss the assessment and appraisal of health-care services and allocation of available resources.

Results At least seven essential variables were identified that should be heeded when applying the concept of QALYs for decisions concerning health-care provision. The efficiency frontier (EF) concept can be used to set a ceiling price and perform a cost-benefit analysis of provision, but different stakeholders—a biostatistician (efficacy), an economist (costs), a clinician (effectiveness), and the patient (value)—

could provide a fairer appraisal of health-care services. Efficacy and costs are often based on falsifiable data. Effectiveness and value depend on the success with which a particular clinical problem has been solved. These data cannot be falsified. The societal perspective is generated by an informal cost-benefit analysis including appraisals by the above-mentioned stakeholders and carried out by an authorized institution.

Conclusion Our analysis suggests that study results expressed in QALYs or as EF cannot be compared unless the variables included in the calculation are specified. It would be far more objective and comprehensive if an authorized institution made an informal decision based on formal assessments of the effectiveness of health-care services evaluated by health-care providers, of the value assessed by consumers, of efficacy described by biostatisticians, and of costs calculated by economists.

Keywords Health-care appraisal · Patient's value · Society's benefit

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Introduction

The original objectives of health-care systems were to prevent illnesses and health-related problems and help those already affected to recover. We seem, however, to have lost track of these original objectives. Gawande (2009) describes the change of the health-care system into a purely commercial business in some parts of the USA. The commercialization of medicine challenging health-care systems everywhere runs counter to traditional medical professionalism (Irvine 2001). Consequently, the European Commission has defined accessibility, quality, and sustainability as long-term objectives for national health-care

systems (http://ec.europa.eu/health-eu/care_for_me/long_term_care/index_en.htm).

These objectives can be achieved if we (patients, physicians, nurses, social workers, insurance companies, and the pharmaceutical industry) reject unreasonable demands and refrain from creating unbeneficial new ones. Some health-care providers claim to optimize health-care delivery (Porzsolt and Kaplan 2006), but actually only drain health-care resources. As a result, these conflicting interests are making the structure of the health-care system increasingly complex.

Politicians are expected to provide a framework guaranteeing equal access and sufficient financial support while avoiding moral hazard and abuse of the system. An overview (Zentner 2005) investigating the framework of decision-making in 11 national health-care systems found it difficult to describe the actual procedures involved and the roles of the different participants, although several countries have developed codified processes to make health-care decisions. Some systems collect data on medical outcomes to support the statistical comparison of medical interventions. The British National Health Service (NHS) provides a quality-and-outcomes framework based on the annual assessment of patients with chronic diseases (<http://www.gpcontract.co.uk>). In Germany, the first analyses of the Disease Management Programs (DMP) are available and indicate room for improvement (Joos 2005; Szecsenyi 2008; Porzsolt 2008a).

Transparency is essential in complex, regulated systems like public health-care systems; it has the same function in a regulated system as competition does in a market-based system (Porzsolt 2009a). Because a lack of transparency in regulated systems increases the risk of ineffectiveness, the Healthcare Forum of the Federal State of Baden-Wuerttemberg in Germany established a project group to perform two tasks: to identify the weaknesses of the available methods (Zentner 2005) for allocation of health-care resources and propose strategies to optimize these methods.

This paper describes the deficiencies of the methods most frequently used to allocate health-care resources, and tools and methods to be developed to optimize this allocation.

Methods

Following a literature search (keywords: public choice, health economics, decision making, evidence-based medicine, health-care policy, expert panels, guidelines, clinical pathways, patient-related outcomes, shared decision-making, health-technology assessment) and discussions with an international group of health-care professionals, we organized

the three following discussion groups: the Clinical Economics Group at the University of Ulm consisting of academic teachers and medical students; practitioners, scientists, and managers invited by the Healthcare Forum of the Federal State of Baden-Wuerttemberg to discuss new concepts for assessment and appraisal of individual and societal health-care benefits; and an international group of scientists who had previously cooperated either in joint projects on evidence-based medicine or on health-related quality of life.

The discussion in all three groups began with the identification of deficiencies in the most frequently used methods—the calculation of quality-adjusted life years (QALYs) and of the efficiency frontier (EF)¹, which is recommended by the Institute for Quality and Efficiency in Healthcare (IQWiG) in Germany. We included the results of a Health Technology Assessment (HTA) report by Zentner et al. (2005) analyzing the methods used to assess the value of health-care services in 11 countries. We then tried to combine the rather controversial ideas and proposals by searching for compromises.

Results

During the preliminary discussions, the deficiencies and weaknesses of the two above-mentioned methods for allocation of health-care resources (QALYs and EF) were summarized. A new strategy based on four different assessments was then proposed. This procedure is both more transparent and practical for reaching everyday decisions than the other methods.

Variables influencing the formal descriptions of health-care value

One of the most frequently used methods to describe beneficial health-care treatment is the calculation of QALYs (Drummond 1997). It is applicable in almost any clinical situation and provides cardinal values (time-adjusted utilities) that can be linked to monetary units, as well as the relationship between time-adjusted utilities and costs, which can be used to allocate resources. The concept and problems of QALYs have been extensively discussed (Robine 1993; Erickson 1994; Benson 1998; Prieto 2003). The method frequently lacks transparency when applied in daily practice. The approaches used for collection of data on utilization include subject interviews, subject surveys,

¹ This describes the monetary cost in relation to any selected effect, e.g., the absence of gastrointestinal reflux or percent reduction in low-density lipoprotein concentration. It is used in the finance sector as a capital-asset pricing model (Markowitz 1999) and occasionally adapted to health care.

provider surveys, reviews of medical records, health-care utilization diaries, and insurance claims (Goossens 2000).

This wide spectrum of methods results in a common denominator, the QALY, which is used as a basis for making decisions concerning the allocation of health-care resources. It is, however, almost impossible to confirm the comparability of data from two different calculations without describing the variables influencing them. In most studies in which QALYs are calculated, quality of life is self assessed, although proxy ratings are also used. Several studies have revealed considerable differences between self assessments and proxy ratings (Geldmacher 2008). Due to these inconsistent findings, some authors recommend excluding proxy ratings when calculating QALYs (Muennig 2006). A survey by Furlong (2005) describes substantial differences among various measurements.

QALYs are not generally accepted by decision makers in all countries for a number of reasons. The German Institute for Quality and Efficiency in Healthcare (IQWiG) prefers to calculate the EF instead of QALYs for the allocation of health-care resources (<http://www.iqwig.de/index.2en.html>) for three reasons: to provide information to the political authority for setting the ceiling price at which a superior health technology in a given therapeutic area should continue to be reimbursed, to address the benefits of treatment relative to costs for a given indication, and to consider health benefits in the economic assessment as published in IQWiG's methods (Caro 2008).

The literature search and our discussion groups identified at least seven variables that influence the description of (patient-related) outcomes, i.e., in quantity and quality of

life and outputs, i.e., surrogates of outcomes. As a consequence, any scientific report that describes the efficacy and effectiveness of health-care services can specify these seven variables. These variables refer to the self or proxy rating, the perspective, the characteristics of the instrument for QoL assessment, the setting in which the assessment takes place, confirmation of the validity of the assessment, application of Bayes' principles (calculating the change relative to the individual baseline instead of using cardinal values), and whether or not the preferences of individual patients are taken into consideration.

Necessary specifications of variables

The necessary specifications of these variables are listed in Table 1. The effects of health-care services can be self assessed or proxy rated. If proxy ratings are used, it should be specified whether the perspective is limited to a single laboratory result or a single functional test. A multidimensional perspective may be provided by assessment of quality of life, medical results, side effects of treatment, and treatment costs. The assessment may be performed by a single researcher or by a multidisciplinary team, including economists, psychologists, clinicians, and patients. Such assessments would represent a much broader view by including both providers and consumers. Several studies have shown that assessments made under the ideal conditions of a controlled clinical trial generate different results than the same trial completed under everyday conditions (Summerfelt 1998; Davidson 2006; Amin 2004; Wolraich 1999). Therefore, it is essential to describe whether the data were generated

Table 1 Variables that contribute to either under- or overestimation of outputs and outcomes, e.g., in the process of computing QALYs. Under- or overestimation cannot be predicted, as it depends on the rater's expectation and on the topic condition being rating

Causes of under- or overestimation of rating results		
Perspective	Proxy rating	Self-assessment
Proxy perspective	Mono- or oligodimensional ^a	Multidimensional ^a
QOL instrument	Specific ^b	Generic ^b
Setting	Ideal conditions, such as in a clinical trial	Everyday conditions
Validity confirmed	No ^c	Yes ^c
Bayes' principle	Considering patients' baseline conditions ^d	Considering cardinal values ^d
Evaluation of results	Respecting individual patients' perceptions	Not respecting individual patients' perceptions

^a A mono- or oligodimensional perspective (e.g., limited to a single laboratory result or an improvement in a single physical function) often prevails in individual disciplines. A multi-dimensional perspective (e.g., quality-of-life-related results, plus medical results, plus therapeutic burden, plus monetary costs) is more common in a multidisciplinary team including economists, psychologists, doctors, and patients, thereby representing the views of both providers and consumers

^b Generic quality-of-life (QoL) instruments are ideal to compare different services, but lack the sensitivity and specificity to detect many differences in treatment results

^c Validity refers both to the rigor with which a study has been conducted and to the general transferability and applicability of its results

^d The same absolute advantage may imply a life-saving difference for a severely ill patient or may be almost meaningless for a nearly healthy individual. Similar objective advantages may receive highly different subjective evaluations from patients with different individual preferences. Increased investments in a given therapy may gain diminishing marginal utility

under conditions similar to those under which the data will be applied. As up to 80 percent of scientific reports lack validity, i.e., overestimate the size of the described effects (Barreto 2005; Atashili 2006; Hallfors 2006; Steg 2007), there is a growing need to apply standardized methods to confirm the validity of reported results (Bombardier 1999; Glasgow 2003; Akobeng 2008). Different groups of patients have different needs and preferences. Therefore, the evaluation of health services, i.e., which of the patient's problems can be solved by a particular service, seems highly important for decision making.

How to avoid formal calculations of the public benefit of health care

As authentic decisions about the value of health-care services depend on the perspectives of the decision makers, the perspectives of several different stakeholders should be taken into consideration. Table 2 contains the perspectives of four stakeholders—scientists (e.g., biostatisticians), payers (e.g., economists of health insurances), health-care providers (e.g., doctors), and health-care consumers (e.g., patients). These four stakeholders make different decisions when assessing the value of health-care services. Therefore, it is necessary to combine their decisions into a single public decision, which can only be done by a legally authorized institution. The details of the organization will differ in different countries (Zentner 2005).

The proposed solution is based on the assumption that the final public decision about health-care provision is too complex to be formalized. Figure 1 demonstrates that complex problems, such as calculation of the bearing capacity of a bridge, require expert knowledge. More

complex problems, like predicting the future course of shares, may be solved as accurately by expert knowledge as by gut feelings (Gigerenzer 2007). Although the final political decisions in health-care provision are rather complex and cannot be made formally, the decisions of various stakeholders that provide the basis for the political decisions may at least partially be reached in a formal way. Table 2 shows that different stakeholders provide different expertise, use different comparators and endpoints for their assessments, consider different settings, and apply different criteria to make their decisions.

Statements by biostatisticians and economists are available for almost any health service, whereas systematic statements by health-service providers and consumers are rare in the scientific literature. The value of health care from the doctor's and the patient's points of view could be documented if doctors and patients explicitly define the problem to be solved by the care provided and the average time interval within which the problem is expected to be solved. Doctors and patients should initially provide statements on the degree to which the defined problem could be solved (completely, partially, barely, not at all, or with new additional problems emerging). We have already started the first projects in which doctors and patients define the main problem, treatment goal, and the parameters that reliably indicate the degree of goal attainment (Porzolt 2009b). The descriptions of the degrees of goal attainment from the doctors' and the patients' points of view can be used as two separate indicators for the overall quality of a treatment. The difficulty of making adequate and equitable decisions about health-care provision becomes evident, as statements that can be falsified a priori are not better than those that cannot be falsified. The final decision about publicly

Table 2 The four dimensions of health-care assessments and appraisals provide the basis for the fifth dimension of societal benefit. This fifth dimension is the result of a democratic decision based on the results of the four previous assessments

	Efficacy	Costs	Effectiveness	Value	Benefit
Perspective	Biostatistician	Economist	Provider	Recipient	Society ^a
Expertise	Preclinical experts		Clinical experts		Decision makers
Compared alternative	Best available treatment	Opportunity costs	Provider's experience	Recipient's experience	Opportunity costs and value ^b
Endpoints	Outputs or outcomes ^c	Monetary costs	Health-care providers may report outputs or outcomes ^c (effectiveness), but recipients should address outcomes ^c (value)		Societal objectives
Setting	Ideal but artificial study conditions		Real-world, everyday conditions		
Criteria for evaluation	Statements that can be falsified		Statements that cannot be falsified		

^a Society has to decide about the mix of market competition and government regulation

^b The perceived value is not independent from the provided information. Both the costs and the provided information will vary among different societies

^c Outputs are defined here by surrogate parameters, such as blood pressure or laboratory results (without confirmed effects on quantity or quality of life), while outcomes represent patient-related outcomes, such as the confirmed reduction of strokes due to reduced blood pressure in a defined risk group

Fig. 1 General hypothetical relationship between required knowledge and complexity of the problem. Rational explanations may be sufficient to solve problems up to a certain complexity. Very complex problems may be solved as efficiently by gut feelings as by evidence-based decisions

financed health care is based on a cost-benefit analysis, i.e., the societal value of health services as related to monetary units. This societal decision can only be made by an authorized institution.

Discussion

Cost-effectiveness research is the comparative analysis of two or more alternative interventions in terms of their health and economic consequences. The results are expressed as an incremental cost-effectiveness ratio, i.e., the ratio of differences in costs between a pair of medical interventions to the differences in the corresponding health effects. This definition is well accepted, but includes neither the patients' goals nor their assessment by the patients (Biswas 2008a, b). The vast majority of everyday decisions in health care are made by health-care providers (e.g., doctors). As various health-care professionals claim the right to make health-care decisions, it seems important to define the necessary qualifications for making these decisions. Some of the emerging problems are not directly related to health care, but to the wider context of health-care provision.

One problem is nomenclature because different disciplines use different concepts or terms for essentially the same thing or the same term for something entirely different. An example is the different meanings of 'efficiency' in pharmacology (Chu 2004), physics, technology, and epidemiology (Last 1988). Within a particular discipline, differences that are not generally recognized may, however, be important. An example is the difference between cardinal and ordinal utility. It is well known that only cardinal utilities can be used for multiplications that

are made in the calculation of QALYs. Awareness of this problem is essential for all stakeholders to avoid mistakes.

A second problem in decisions about the allocation of health-care resources is the translation of decisions into everyday practice. Practitioners, ethicists, and other health-care professionals have to be aware of the limited validity of a considerable portion of the scientific literature (Bailey 2004; Hailey 2004; Howley 2008). Critical comments made by various stakeholders, the systematic documentation of treatment goals, the time within which the desired response is expected, and the observed treatment outcome (from both the physician's and the patient's perspective) should be introduced into day-to-day practice. This is easier said than done, as our usual strategies address the structure and the process, but not the intended outcomes achieved by health-care services.

The cursory understanding of the QALY concept creates a considerable risk of drawing wrong conclusions and consequences in health-care decisions. Experts (i.e., those who calculate and publish data on QALYs) are usually aware of these problems, but do not always take the necessary and adequate limitations into account when interpreting QALY results. A second problem is that the majority of users (i.e., those who don't calculate but use results expressed in QALYs) have only vague knowledge about the limitations of this concept. Therefore, more transparency is needed when reporting results on health-care assessments.

This publication suggests a new approach that may apply to different health-care systems, as it addresses the conflict between the individual patient's value judgment, which is advocated by some lobbyists, and societal benefit, which is publicly advocated. The method we propose to settle this difference is based on four steps. First, the weakness and lack of transparency of one of the most frequently used methods for allocation of health-care resources has to be described. Second, four stakeholders are requested to provide a formal evaluation of the health-care service in question from their own perspectives using their preferred endpoints, conditions, and dimensions. Third, the formal evaluations of the four stakeholders are presented to an authorized institution. Fourth, this institution has to convert the evaluations from the four specialist fields into a societal consensus.

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