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Current controversies of using cost per QALY for cost-effectiveness analysis-Isn't two decades enough?

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Abstract

Cost per quality-adjusted life year (QALY) has murky origins and is empirical by nature. The fundamental issue is that QALY is not aimed at valuing health improvements but rather valuing health states. In the present era of breakthrough innovations and personalized medicine, the cost per QALY approach is outdated and far too imprecise.

Cost per QALY remains a golden standard for some health technology assessment agencies to determine the value for money of innovations. The method is associated with well-acknowledged shortcomings. Criticism to cost per QALY is steadily growing and echoed by experts and international organizations.

Cost-effectiveness analyses are expected to be expressed as costs per relevant clinical outcome and integrate fairly all relevant attributes. Cost per QALY assessment for health decision-making played its role in the last decades but should be abandoned in light of the current knowledge and nature of new medical technologies.

Introduction

Pharmaco-economic modelling is assumed to be based on the fundamentals of probability inequalities introduced in 1962 by Hoeffding [1]. In 1997, Weinstein et al. [2] proposed the incremental cost-effectiveness ratio (ICER) per a quality-adjusted life year (QALY) in health care. Since it was first introduced, cost per QALY has been referred to in tens of thousands of publications, but only in a few concerns regarding QALYs have been previously described, yet not fully rejected [3]. Meanwhile, medicine moved from incremental innovations to breakthrough therapies, and amid these transformations, the controversy around cost per QALY's background merits examination.

Background of cost per QALY

The origins of this method are perceived as "murky" [4]. Health care professionals warned that the use of QALYs may lead to "absurd" anomalies [5]. Nonetheless, cost per QALY remains a golden standard for some health technology assessment agencies to determine the value for money of innovations.

A fundamental problem in the use of cost per QALYs is its empirical nature. QALY is not aimed at valuing health improvements but rather at valuing health states. The current framework of economic evaluation only considers health-related preference-based outcome measures; as such, the concept of utility as measured by QALYs is much narrower compared with that in economic theory, in which welfare refers to all services that provide individuals with utility. As Harris wrote as early as in 1991, "It is lives that are valuable and not life-years" [6]. This point remains and always will be valid. In the conventional concept of QALYs, a health state that is more desirable is more valuable. A critical question is, desirable to whom [7]? Certainly, often not to patients.

Criticism to this methodology has been growing [8-11], and concerns about its limited capabilities to address adequately health determinants and assess satisfaction of society needs are echoed by international institutions [12-14]. After decades of incremental innovations, medicine has finally reached breakthrough stage; the numbers of target therapies and personalized medicine, including stem, gene, and cell therapies, are steadily rising. Treating cancer and rare and congenital diseases achieved unprecedented results. The value of medicines needs reconceptualization. Value, which is neither an abstract ideal nor a code word for cost reduction [15], encompasses new frontiers of efficiency involving numerous interventions and practically integrated health care approach. As the public sector plays a disproportionately large part in funding health interventions [16], paying the main fraction of total pharmaceutical expenditures, governments become sole decision makers in remunerating or discouraging innovation. New math on drug cost-effectiveness [17] is urgently needed. The use of value-based pricing and multi-criteria decision analysis [18] can be the first steps to properly assess the value of health care, finally bringing patient-centeredness and empathy into the process. Failure to act with haste will deteriorate the cumulative socio-economic inequalities in the allocation of health resources [19-21]. Heterogeneity in estimates on willingness to pay for QALY is well known [22]. Lastly, the results of the EU- funded European Consortium in Healthcare Outcomes and Cost-Benefit Research (ECHOUTCOME) experiment established that health assessments expressed in number of QALYs or cost/QALY are inconsistent and can lead to divergent results, because the underlying assumptions of the QALY model are not validated [23].

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Discussion

Many outcome researchers increasingly ask the question: What would or could replace QALY [24]? First, all nine attribute domains of the European Network for Health Technology assessment (EUnetHTA) HTA Core Model® [25] should be finally integrated by the HTA decision framework. Multiple criteria decision analysis (MCDA) for health care decision-making is an appropriate approach for integrating multiple attributes [26,27]. The constraint optimization model has also attracted increasing attention; it offers maximization of population health gain to predefined and recognized constraints [28]. Health outcome-based payments, most often seen as pay-for-performance (P4P), are already used in Europe, the US, and other countries [29-35].

Reliance on a cost-effectiveness based on cost per QALY gained ignores the actual needs of patients, especially in the light of breakthrough therapies. This method does not properly account for benefits. Empirical by nature, the cost per QALY approach fails to distribute health resources in the fairest way within society. Proper solutions available need to be secured to allow access to novel therapies.

Conclusion

- Cost per QALY assessment for health decision-making played its role during the last decades but should be abandoned in front of the current knowledge and nature of new medical technologies.
- Cost-effectiveness analyses are expected to be expressed as costs per relevant clinical outcome and integrate fairly all relevant attributes. The use of case-by-case modelling, instead of the progressive replication of a methodology that is organically associated with well-known bias and uncertainty, in the future is not unfounded.
- It is high time for health systems to adopt empathy to patients and start discussing opportunity costs gained by a novel technology and its societal perspective.

As integral medical care, personalized medicine and advanced therapies are here to stay, and the role of ICER per QALY becomes more imprecise than ever. Valuing such new technologies by cost per QALY is outdated. Indeed, doing so may be considered a scientific misconduct, blinded to patient centeredness and societal perspective. It is difficult to find excuses for the continued wide use of this approach in the era of breakthrough innovations. In the next five years, MCDA and P4P will inevitably replace cost per QALY. However, why wait so long? QALYs do not fully reflect the outcomes of all relevant medical services. Outcomes-based market entry agreements are most useful when there is uncertainty in clinical or economic outcomes, while switching the risk entirely to manufacturers.

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