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Notions of "Value" in Healthcare

Future of Patients in Healthcare Evaluation: The Patient-Informed Reference Case

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ABSTRACT

The "Reference Case" was developed to facilitate comparability among published cost-effectiveness analyses intended to contribute to decisions about the broad allocation of healthcare resources. Although the societal perspective is recommended for Reference Case analyses, empirical estimations rarely adequately represent the patient perspective, and more often, healthcare system or payer perspectives are used. In this commentary, we discuss the evolution of the Reference Case over the past 20 years and how it now needs to further evolve. This should begin with a patient-informed societal perspective. A realignment of the societal perspective to better include patient perspectives in CEA creates a conduit for patient inclusion. Engaging patients to both derive patient-informed value elements and prioritize value elements using stated preference methods will lead to patient inclusion in the societal perspective and a patient-informed Reference Case analysis.

Keywords: patient perspective, Reference Case, societal perspective

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Introduction

More than 20 years ago, guidelines for cost-effectiveness analysis (CEA) were developed in response to a growing need for economic evaluation of new healthcare technologies. The seminal US Public Health Service Panel on Cost-Effectiveness in Health and Medicine (PCEHM)[1] recommended the "Reference Case" to facilitate comparability among published CEAs with the intention to contribute to decisions about the broad allocation of healthcare resources. The Reference Case reflects the societal perspective, a comprehensive viewpoint that encompasses the range of costs and outcomes of concern to all members of society.[1] In this commentary, we discuss the evolution of the Reference Case over the past 20 years and how it now needs to further evolve into the future. With recent attention to patient perspectives in economic evaluation,[2,3] and more specifically in the United States, we now focus on patient engagement in attribute development for use in value assessments.[4-6] This represents the foundation for the advancement of patient-informed economic evaluation and addressing the existing tensions in perspectives used in CEAs for healthcare decision making in the United States. A return to the societal perspective is needed, with an emphasis on the patients who comprise society.

The Use of Perspectives in Economic Evaluations

When the First Panel published its guidelines for conducting CEA in the United States, the societal perspective was chosen for the Reference Case because it was intended to represent the public interest rather than that of a specific stakeholder.[1] Although it was intended that the patient perspective was embedded in that of society, patient-specific measures, such as productivity gains and losses, began to disappear from evaluations, and the societal perspective, as it was originally defined, was inadequately applied to CEAs. There appeared to be a mismatch in the academic guidance of CEA methods (ie, that the societal perspective was the recommended Reference Case) and the preferences of those using CEAs in decision making (ie, that a payer perspective was more practical).

Over time, CEAs strayed from the First Panel's perspective recommendations and instead used a healthcare sector or payer perspective for their analyses,[7] partly by necessity. For example, the International Society for Pharmaceutical Outcomes Research (ISPOR) Drug Cost Task Force Report described the difficulty in measuring drug costs from a societal perspective.[8] Even in countries where CEA was being used more ubiquitously in decision making, the societal perspective was generally not adopted.[9] In the aforementioned ISPOR Report, Garrison et al. described that

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the societal perspective is grounded in normative economics and gives decision makers a means to compare what should be done in the healthcare marketplace.[7] The authors discussed complications of drug costs from the societal perspective and put forth a number of recommendations, including that CEAs take a "limited societal" or "health system perspective."[7]

More recently, the Second Panel report of 2016 recommended that CEAs report both a Reference Case analysis based on a healthcare sector perspective and one using the societal perspective.[9] This guidance responded to researchers' growing use of the healthcare perspective and maintains the validity of the societal perspective, but it may not necessarily encourage its use as a tool for decision making.

The US Context of CEA and Value Assessment

Although the publication of CEAs in the US literature has increased dramatically over the past 2 decades,[10,11] the United States has not used CEA in healthcare decision making to the degree that it has been used elsewhere around the world. When the First Panel published its report in 1996, the use of CEA was growing in other countries, notably the United Kingdom, for informing coverage and reimbursement of healthcare technologies. The slow adoption of the use of CEA in the United States may be attributed to a number of causes. The fragmented US healthcare system comprises public and private payers, and they make their own decisions about value and what treatments they will cover. Therefore, their use of CEA for decision making is inconsistent. For example, Medicare uses CEA to make decisions about prevention but not treatment.[12] Neumann et al., in their comprehensive discussion of the topic, also cited the country's "cultural and political traditions that emphasize personal and economic freedoms."[9] It is perhaps a combination of these factors that has sparked a discussion on the need to reflect the patient's perspective in the recently developed value assessment frameworks, of which several include the use of CEA.[2,3,13,14] It is important to incorporate patient views into other aspects of value frameworks, but if not included in CEA specifically, they are easily ignored during decision making.

Value assessment in the United States has faced recent criticism for its lack of patient inclusion.[15,16] The scientific community has responded with an interest to focus on patients and a discussion of appropriate methods for inclusion of patients' views. Policy makers will need more guidance on the process of patient involvement. We believe that a Reference Case using the societal perspective, once the theoretical underpinning of CEA-based economic evaluations, provides a basis for patient inclusion. In the past, the societal perspective was applied without understanding what patients actually experienced or cared about in terms of outcomes and costs. A more patient-informed societal perspective would appeal to US society's value of individual attributes and preferences. What is needed now is a re-examination of the societal perspective that better focuses on patients so that it can strengthen CEAs within the framework of a Reference Case.

Realigning the Societal Perspective to Reflect Patient Values

Recently, the issue of perspective has come into focus because CEAs are being conducted as part of value assessment to be used by payers and stakeholders such as manufacturers, patients, and patient groups. CEA methods are being critically examined for their ability to contribute to accurate value assessments that are expected to be used by payers and providers to assess value of healthcare on behalf of individuals and patients. The challenge is that the perspective taken in economic evaluations, typically that of the healthcare sector, may not align well with that of the patients who enroll in healthcare plans and use healthcare services. As the current use of value assessment in the United States evolves, payers will need to better reflect patients' values in the evidence they use for healthcare coverage decisions.

There have been a number of methodological recommendations for reflecting patient values in both value assessments in general and CEA specifically. The ISPOR Special Task Force recently addressed the existing value frameworks' approaches and methods. They suggested that "population-level decisions can be more patient centric" with value elements that capture patient preferences or utilities.[17] Recently, others have also voiced a need for value assessment frameworks to better account for the patient perspective by including decision-making attributes important to them.[18,19] Patient engagement does not necessarily lead to completely individualized CEAs that are no longer designed for population-level decision making. Rather, an expansion to traditional CEA has been suggested. In particular, a broadened view of value was proposed with a number of novel value elements described.[17,20] A patient-informed Reference Case would address the need for evaluations to reflect patient values by formally recommending this approach in CEA methodology. Once patient-specific value elements are defined and measured, updated guidance for Reference Case analyses could include them. Although CEA is just one piece of value assessment, a formal structure to patient inclusion could lead to greater uptake of CEA findings in healthcare decision making. A realignment of the societal perspective Reference Case to better include patient perspectives in CEA creates a conduit for patient inclusion.

Patient-Informed Value Elements

Work to further derive and examine patient-informed value elements (ie, value elements that are expressly identified and defined by patients) is critically needed and will be the basis for their inclusion in the Reference Case. We must start by identifying the outcomes and attributes of healthcare that are valued by patients. Deriving attributes of patient value is the first step toward incorporating these into economic evaluations. As described recently, stated preference methods can be used for this derivation.[4,6,21,22]

We have begun to examine patient-informed value elements using an iterative process of eliciting elements and definitions from patients and the patient community (ie, patients, families, family caregivers, patient advocates/groups); understanding their relative importance in groups of patients with specific conditions; quantifying the utility for the derived value elements, and aligning this with economic evaluations. Our preliminary work reveals that some elements important to patients are already incorporated in CEAs using a societal perspective such as out-of-pocket costs, caregiver time costs, transportation costs, or effects on future productivity. Such elements may be considered patient-informed value elements if the values themselves are derived from patients or if their importance and prioritization is derived by engaging patients. By eliciting value directly from patients, we can assess whether the importance of these elements aligns well with the methods traditionally used to estimate them and the uncertainty in these estimates.

Patient value elicitation may also reveal novel value elements that were omitted from previous evaluations or for which we presently have no measures to reflect these elements in CEA, such as effects on family members. Our current work has revealed that

ability to plan and consistency of care or, in the case of communicable diseases, concern for their community, are important to patients when making treatment decisions. Methods for incorporating value elements into CEAs and other economic evaluations are needed. Lakdawalla et al. provided quantitative guidance on how to incorporate recently proposed novel value elements.[20] Some may be disease specific and some may span many conditions. More work in this area is needed to create a basis for a better patient-informed Reference Case.

Recently, there has been much discussion about existing methods for valuing health states[23] using generic preference instruments[24] for quality-adjusted life-year (QALY)[25] estimation. Patient value elicitation may reveal that instruments used to measure health state utilities do not adequately measure changes in health states for particular patient groups or conditions. In fact, there are data showing that stated preference methods, like a discrete choice experiment, render important information about treatment value to which traditionally derived QALYs are not sensitive.[26] For example, the value sets for preference instruments can be estimated with discrete choice experiments.[24] Furthermore, it is important to examine whether the domains measured by instruments used for QALY estimation reflect attributes of interest to patients. This particular topic is perhaps at the heart of the current tension in the United States surrounding the use of value assessments for decision making. The debate often cites that patient values are in contrast with population-level decision making and that under budget constraints, we must make a decision for the "average patient"; otherwise, considering novel value elements for some patients will result in reduced benefit for the rest of the population. Or vice versa, population-based estimates are being use to make decisions are being used to make decisions about individuals. We believe that patients are important stakeholders among others (ie, payers and manufacturers). We are focusing efforts on understanding heterogeneity in patient preferences and how we might describe value for subgroups based on values and preferences.

Continuous patient engagement has a role not only in deriving value elements, but also to inform CEA model assumptions and structure.[27] The individuals comprising the US population make decisions about what health plan they choose, and if they enroll in a plan and later become a patient, how they make decisions about treatment options covered by that plan. Therefore, patient views must be reflected at the core of the societal perspective. To date, CEA methods do not explicitly consider the patient an important stakeholder. Future economic evaluations should not only include the patient's preferences and perspective in the Reference Case, but could also include patient input into model-based CEAs. Engaging patients in the process of economic model development as research partners will ensure that research methods, model structure, and parameter inputs reflect patients' values and produce a model and resulting evaluation that form the patient-informed societal perspective. Patient engagement during model development helps inform structural decisions such as health states included or time horizon.[28] For example, a transition-model diagram may contain one state labeled, "symptomatic," which may really be composed of multiple states reflecting symptoms with greater severity. By asking patients to describe their journey through diagnosis and treatment strategies, model structure could be adjusted to better reflect their true experience. These specific approaches would provide a basis for patient inclusion in the societal perspective and a patient-informed Reference Case analysis.

Paving the Way to Patient Inclusion: The Future of the Reference Case

The growing emphasis on the patient perspective indicates a need to better reflect the patient perspective in value assessments. As the Reference Case has evolved to suit the needs of decision makers, we have strayed from the societal perspective and forgotten that "society" is made up of patients. As the field has brought light to the importance of the patient perspective in developing economic evaluations, there appears to be a growing need for a patient-informed Reference Case analysis reflecting the patient perspective. The basis for this lies in rigorous methods to not only derive patient preferences and value elements, but also include them in economic evaluations. Including patients and patient groups as research team members is the first step. Patient engagement in clinical research and medical product development will allow future value assessments to include data that are already patient centered. The Patient Centered Outcomes Research Institute in the United States has moved the field forward by engaging patients in comparative effectiveness research. We now have methods and emerging guidance for meaningful patient engagement in research and product development, and the next challenge is to bring this fully to CEA. We have summarized several key considerations for the proposed Reference Case in Table 1. A

Table 1. Proposed key considerations for implementing the patient-informed reference case.

Preliminary work reveals four distinct ways to implement the patient-informed Reference Case	
Patient-Informed Elements Existing in the Societal Perspective • Costs such as out-of-pocket costs, caregiver time costs, transportation costs, or effects on future productivity are already described in the societal perspective Reference Case. • Patient-informed adjustments include deriving input values or prioritization by engaging patients. • The importance of these elements should align with the methods used to estimate them as well as the uncertainty in these estimates.	Novel, Patient-Informed Value Elements • Patient value elicitation reveals novel value elements for which we have no measures to reflect these elements in CEA (eg, ability to plan, convenience, effects on family.) • Elements may be disease specific or common to many conditions. • Research is needed in this area.
Valuation of Health State Utilities • Examine whether the domains and attributes used for QALY estimation reflect those of interest to patients. • Consider alternative approaches such as stated preference methods (eg, DCE) to value health state utilities.	Patient Engagement in Economic Evaluations • Patient input into model-based CEAs. • Model structure will better reflect patient journey through treatment strategies. • Patients help inform time horizon and modeled health states.

CEA indicates cost-effectiveness analysis; DCE, discreet choice experiment; QALY, quality-adjusted life year.

patient-informed societal perspective for use in the Reference Case could pave the way for patient-informed CEA as a part of value assessments for healthcare decision making.

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