



Patient-reported measures: how useful in health economics?

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Introduction

There is a growing interest in patient-reported measures to assess the various dimensions of health and health care [1]. Indeed, it is commonly accepted that individuals do not only value the survival benefit associated with an intervention or a treatment but also his/her health state and the quality of life he/she will experience. Patient-reported outcome measures (PROMs) have been developed and are frequently used in the assessment of new health technologies [2–4]. More recently, there has been interest in the patient experience, the underlying idea being that outcomes are not the only thing that matters. Patient-reported experience measures (PREMs) aim to capture how the patient went through the process of care. PREMs were initially developed to measure the quality of care. The question we wish to highlight here is whether or not patient-reported measures (and more specifically PREMs) are useful from health-economic point of view.

What are PROMs and PREMs?

PROMs are self-reported instruments on the status of a patient's health condition from the patient himself without the interpretation of the patient's response by a clinician or anyone else [5]. PROMs include various dimensions such as symptoms, functioning, emotional state, psychological state, social role, spirituality, etc. These dimensions describe the

quality of life, and more precisely health-related quality of life. There is a multitude of validated instruments. PROMs may be generic and apply to any subject or any disease (e.g. SF-36 or the Nottingham Health Profile) or disease-specific (e.g. QLQ-C30 or FACT in cancer) or specific to a treatment (e.g. FACT-BMT for bone marrow transplantation in cancer patients). Specific scales can be found for a wide range of acute and chronic conditions. A third category of PROMs comes from the field of economics and measures patients' preferences.

PREMs are a measure of the patient experience regarding the care he received. These measures usually cover satisfaction with care, waiting times, cleanliness of facilities, information received, relationships with the healthcare professionals and interactions with staff in general. These tools differ from pure satisfaction and intent to report objective patient experiences removing subjectivity [6]. Kingsley et al. distinguished two categories of PREMs according to the nature of the items considered which may be mainly relational (e.g. being listened to by professionals) or functional (e.g. facilities available). The number of PREMs instruments is much more limited than that of PROMs. Reliability and reproducibility of available instruments are a limitation for their use [7]. The Consultation and Relational Empathy (CARE) questionnaire is a relational PREM to measure patients' perceptions of relational empathy in the consultation in primary care [8]. Similarly, the Canadian Health Care Evaluation Project Questionnaire (CANHELP-lit) is another example of relational PREM that includes a patient version and a family caregiver version. It focuses on the relationships with physicians and nurses and the satisfaction with end-of-life care [9].

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How are they used ?

The use of patient-reported measures in health care can be categorized according to the end purpose of the measure and its recipient (patients, decision policy makers and/or health care providers).

Using PROMs to enhance the efficiency of health technologies and the quality of care

A first use of PROs is the assessment of the benefits of new drugs and health technologies within the so-called risk-benefit assessment. Clinical trials increasingly incorporate PROs to measure and compare the quality of life of patients between the trial arms [10]. Second, PROs such as preference-based measures are used in economic evaluations to guide reimbursement decision-making. PROMs are thus a heterogeneous family of tools that include both psychometric instruments for measuring the quality of life and multi-attribute utility indexes that measure individuals' preferences for relative health states. Lastly, PROMs have been used in national programs to compare healthcare provider performances in routine practice as for example in England for common surgical procedures [11, 12] and in Belgium [13].

Using PREMs to enhance the quality of care and the efficiency of the health system

In daily practice, PREMs can be used to get patient feedback during the process of care. It improves the communication between the caregiver and the patient. PREMs highlight on the relationship between those who deliver the care and those who receive it [14]. PREMs may also be used to assess new organizations of care. For instance, in France, a national program called Article 51 aims at funding innovative projects that promote coordination, group practises and the integration of care [15]. Projects must include an evaluation among which it is recommended to incorporate PREMs. Another goal of using PREMs is to improve the quality of care across healthcare providers [16]. Using such tools on a broad scale tends to reduce the variability of practices. PREMs are also used to adjust payments to hospitals within prospective payment systems (PPS). Indeed, it has been shown that PPS may be detrimental to the quality of the care [17]. Thus, in the US where DRG-based payments were first implemented, corrective mechanisms were sought to counterbalance the pitfalls of PPS. Medicare has experimented payment for performance based on process measures with bonus and malus incentives [18]. Finally, both PROMs and PREMs may be useful for international comparisons of health systems. The Patient-Reported Indicator Surveys (PaRIS) initiative from OECD was launched in 2017 with

the aim of providing validated, standardized, internationally comparable patient-reported indicators [19].

Though, there is a consensus on the usefulness of PROMs and PREMs to improve the quality of care and that some PROMs are preference-based measures used to build QALYs, it is unclear whether or not PREMs may be useful for economic evaluation.

Patient-reported measures in economic evaluation

PROMs in economic evaluation

Within the framework of the economic evaluation, effectiveness is often measured in terms of quality-adjusted life years (QALYs). This QALY is a measure of survival weighted by a coefficient that expresses preferences for a given state of health (utility value) in comparison with perfect health. To reveal preferences and estimate utility values, there are direct methods and indirect methods. Direct methods are: the standard gamble based on the theory of expected utility and the time trade off which comes from the utility theory of Hicks or discrete choice experiments which comes from the theory of random utility. For all these methods, some reluctance and acceptability problems from the clinician side may be encountered. Indeed, proposing choices to patients on the valuation of their state of health while they are going through their illness and treatment may raise ethical issues. Indirect methods lie on multi-attribute health status classification systems in which the patient fills the questionnaire to describe his health state and a value joined with the tool provides the utility value corresponding to this health state. Indeed, for this kind of tools, a valuation study is conducted separately interviewing persons from the general population to value all the possible health states (way of filling the questionnaire). Several indexes of this type can be cited as, for example, HUI2/3, EQ-5D, QWB, SF-6D or EORTC-8D, AQL-5D. The EuroQol EQ-5D is the most frequently used questionnaire as it is generic whereas EORTC-8D and AQL-5D are condition specific. This very simple tool recommended by health agencies has led to much more frequent use of cost-utility analyses in routine practice and the adoption of cost per QALY as the reference metric.

PREMs in economic evaluation

(a) Process utility

From an economic point of view, this emerging notion of experience of care from the patient perspective refers to "process utility". Process utility encompasses other characteristics of health care beyond "health" within the patients'

utility function [20]. Considering only health outcomes in the patients' utility function and neglecting process utility in cost-utility analyses could lead to suboptimal provision of health care [21]. Some authors already added process utilities into cost-utility analyses [22]. However, as noticed by Brennan and Dixon [22] two methodological issues need to be considered. The first issue is the period used in the preferences elicitation questions. Identifying a disutility associated with a short time health care intervention needs to be applied to this short time. One can question the relevance of applying short-lasting disutilities to chronic health states [22]. The second issue is about double counting. If patients are already implicitly considering the added therapeutic benefits of process utility in their valuation of health outcomes, then double counting will occur.

(b) Are PREMs and PROMs independent?

The issue of independence between PROMs and PREMs is crucial to guide the decision rule of including PREMs to the numerator or denominator of the cost-utility ratio. Black et al. have shown that there is a weak positive correlation between experience and outcomes and that patients can distinguish between clinical effectiveness, safety and their experiences [23]. In a prospective study among patients undergoing surgical procedures, Black and al found that "Patient outcomes can increase patients experience ratings by 10%, similarly improving patient experience ratings will cause a 3% improvement on outcome scores". Thus patients may consider process utility in their valuation of health state and consequently PREMs are included in part in the denominator of the cost-utility ratio. Similarly, in a systematic review of the literature on the value associated with convenience in health care delivery, Higgins et al. [24] noticed that "A preference for convenience-related process utility exists, independent of health outcomes". One can translate in the existence of PREMs independently of PROMs.

(c) PREMs and CUA

Actually, PREMs measure independent dimensions such as relational and functional aspects and the issue of how to value these aspects for economic evaluation are still debating. Higgins et al. noticed the "high proportion of studies that use a DCE methodology, using some kind of financial attribute to derive a WTP estimation" [24]. This methodology may be used to incorporate process utility (and thus some PREMs) in economic evaluation. However, while it is relatively easy to include process utility in cost-benefit analysis, it is much more debatable how to incorporate process utility in the cost-utility framework. Considering independence between PREMs and PROMs, a way to include PREMs in the CUA (in a welfarist approach) is to consider them as

costs for the patient during the process of care and incorporate these costs in the numerator of the cost-utility ratio.

If process utility is incorporated routinely into economic evaluation, the effects on the allocation of resources in the health care sector are unknown and will depend on the relative size of patients experience dimensions in comparison to other dimensions traditionally considered. Future research in this area is needed and methodological issues for incorporating 'process' into the QALY should be developed in the next years.

Conclusion

Obviously, PROMs like the EQ-5D are used to assess the value of the health gain in the calculation of the cost-effectiveness ratio, i.e. in the denominator (QALYs). It is debatable if and how PREMs may be useful in the framework of economic evaluation. Should they be considered in the numerator to account for the costs borne by the patient while awaiting for care and/or should they be used in the measure of effectiveness considering that procedure has consequences on the patient well-being?

Data availability Not applicable.

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