What Should Be the Role of Patient Preferences in Making Health Care Resource Allocation Decisions?

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(This article was based on a panel discussion on the Issue Panel, “What Should Be The Role Of Patient Preferences In Making Health Care Resource Allocation Decisions?” at the ISPOR 12th Annual European Congress, May 26, 2009, Paris, France, with panelists Deborah Marshall PhD, Canada Research Chair, Health Systems and Services Research, Associate Professor, Department of Community Health Sciences, University of Calgary, Calgary, AB, Canada; JohnFP Bridges PhD, Assistant Professor, Health Policy and Management, Johns Hopkins Bloomberg School of Public Health, Baltimore, MD, USA; Mark Sculpher, PhD, MSc, ISPOR 2010-2011 President-elect and Professor of Health Economics, Centre for Health Economics, University of York, York, UK; and moderated by Michael Drummond, DPhil, Professor of Health Economics, Centre for Health Economics, University of York, York, UK)

Patient engagement is increasingly becoming a formal component of health technology assessment decision frameworks. But consensus has yet to emerge on how and which patients to include. An Issues Panel discussion at the 2009 ISPOR 12th Annual European Congress highlighted the tension between the needs of a society to allocate health care dollars and individual patients’ needs for treatments. There are three main issues surrounding incorporating patient preferences: whether patients’ preferences should play a bigger role in resource allocation decisions, how to accurately measure preferences and whether patient preferences differ from providers or the community at large.

Traditionally, most of the information we have about efficacy of treatments comes from randomized controlled trials. However, these trials do not account for a myriad of factors that influence whether a patient and his or her doctor select a given treatment option [1]. Dr. John Bridges argued individual patient preferences for treatments should be taken into account over the entire spectrum of medical care, ranging from developing new technologies to assessing treatment effectiveness. He argued the traditional process causes manufacturers, physicians and even the health system itself to focus on outcomes that may not be the most important to a patient actually dealing with the disease in question. For example, most literature posit that negative side effects are the culprit in poor adherence to anti-psychotics in schizophrenics. However, a small study using a conjoint analysis (asking participants to rank medication attributes and effects in importance) showed patients with schizophrenia actually valued how clearly they could think and interact socially more than concerns over side effects [2]. If patients’ attitudes toward risk were taken into account, then more new drugs for rare conditions could be brought to market. Patients with serious conditions may be more willing to risk a higher possibility of death from a new drug, in exchange for the hope it may be effective. Limiting new technologies to very low risk levels makes sense on a population basis for a regulatory body, but this process inherently limits access.

The tension between limiting resources and expanding access to care has particular importance when considering patient preferences as part of the allocation decision. In budget-constrained health care systems, there are patients who gain but also those who lose as services are displaced from the funding of new technologies. Dr. Mark Sculpher cautioned that all patients should be taken into account not just those using or benefiting from a particular technology. He said it is challenging to reflect the true array of patient preferences in a constrained health budget. One method advocated by Dr. Sculpher is to base certain allocation decisions on sub-group analyses. Arguably, there are some groups who stand to gain more from certain interventions, but if it is a small group, the treatment in question may not be funded as the cost-per-quality adjusted life year gained will be above whatever threshold agencies have set for incremental cost-effectiveness [3]. The aim for decision makers is to allocate resources to interventions that reflect both the health and other forms of benefit achieved.

Stated preference methods are being used increasingly as more accurate assessments of patients’ preferences than the common health-related quality...
of life measurements. Stated preference methods have a structured and quantitative approach consistent with theory and can consider attributes related to both processes and outcomes. One study examining patient preferences in Crohn’s disease treatments found patients were more willing to accept higher side effects for improved outcomes [4]. This kind of information can have huge impacts on utilization, especially when trying to encourage preventative care. Dr. Deborah Marshall cited her colorectal screening study from Canada, which showed that screening uptake would increase if all testing modalities were offered to patients instead of one alternative [5]. In a separate study, she also illustrated how providers’ and patients’ preferences differ for colorectal screening tests. Patients were willing to pay somewhere between 1.5 and 4 times more for colorectal cancer screenings and were more concerned with test accuracy than physicians expected [6]. Dr. Marshall argued that if we want to maximize up-take of appropriate health care interventions, we need to better understand the choices patients make with stated-preference studies like these. These methods can also allow researchers to measure the rates of substitution between various attributes of a treatment process and calculate patients’ maximum acceptable risk or willingness-to-pay.

In negotiating this space between allocating scarce resources and allowing access to needed treatments, Drs. Bridges and Marshall think a possible third way forward would be to allow for increased use of co-pays. While centralized agencies may decide not to fund a new technology or only fund it to a point, co-pays would allow patients access to the technologies if they can afford it. Dr. Sculpher cautioned this can become an equity issue which could obstruct the goals of population health if used incorrectly. Despite the differences, all three researchers agree that patients should play a bigger role in evaluating technologies in the future.

REFERENCES

Thoughts on the Validity of a New QALY Estimator

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(A response by Benjamin Craig follows this article)

INTRODUCTION
Quality-adjusted life years (QALYs) are the most widely used measure of health in the economic evaluation of health care. They are intended to reflect preference toward health. However, problems with aggregating better-than-death (BTD) and worse-than-death (WTD) health states in general population studies have led to unorthodox approaches to QALY estimation, where between 26% and 34% of responses are adjusted by the researchers [1, 2]. These approaches may contribute to differences between QALY utilities and conjoint analysis estimates for health. The ISPOR workshop, titled “Revisiting the estimation of QALYs” led by Benjamin Craig and co-authored by M. Oppe and J.J. Busschbach at the ISPOR 14th Annual International Meeting, Orlando, FL, USA, May 2009 addressed the problem of aggregating better- and worse-than-death TTO responses to estimate health state utility. The workshop was notable for its effort to make QALY estimation consistent with QALY theory and to unify preference scales (QALY utilities and conjoint analysis). With respect to these concerns, the workshop was constructive in highlighting current deficiencies in QALY estimation. Further, the workshop was a refreshing departure from work that seeks to discredit rather than unify measurements obtained by different approaches. There are, however, two concerns with the new estimator. First, one of the recently developed estimators presented by the authors, the episodic random utility model or ERUM, while in some ways is more consistent with QALY theory than previous efforts, applies a stochastic choice model to judgment data. Second, the ERUM estimator does not distinguish utility conditioned on better-than-death and worse-than-death responses. In this essay, we reflect on the problem of aggregating better- and worse-than-death responses under the ERUM estimator.

QUALITY-ADJUSTED LIFE YEAR ESTIMATION
Notation & Assumptions
We first give some preliminary notation that facilitates discussion of QALY estimation in the presentation. Health profiles (q1,t1; ... ;qntn) reflect a sequence of health state,q, and duration, t, pairs over some time horizon, t1 + ... + tn = T*. For example, if we set the time horizon, T*, equal to 10 years, a typical health profile might be (q1,11; q212 ; q313) = (“full health, 3 years”; “back pain, 6 years”; “wheelchair, 1 year”). Persons have preferences over health profiles. Quality-adjusted life years use mathematical formulae to represent the empirically observed preferences. The most basic QALY model (and the one relevant for the current workshop) assumes that for any health profile (q1,11; ... ;qntn) there is a utility function U, that represents preference over health profiles:

\[ U(q_1,t_1; \ldots ; q_n,t_n) = H(q_1,t_1) + \ldots + H(q_n,t_n). \]  

Note that in Equation 1, utility is linear in time and different time periods contribute additively to overall utility. Usually (but not always) expected utility is used to establish a utility function for QALYs. Expected utility theory hypothesizes that U represents preference in the sense that if we empirically observe that one health profile is “at least as preferable” as another, then utility for that profile is at least as great as it is for the other. Likewise, if we claim the utility of a profile is at least as great as another, then we expect to empirically observe that the health profile is “at least as preferable” as the other profile. We call the aforementioned relationship between preference and utility the expected utility hypothesis.

Under the episodic random utility model, the decision maker is assumed to select (with error) a choice so as to maximize utility. Thus, for individual utility functions over health profiles, like those in Equation 1, a choice probability (the probability of selecting one profile over another) is a function of the differences in utility between the choices and error. Standard random utility models treat the value of attributes as linear in their parameters. Thus, a linear regression in these models is often taken to represent the value of prospects.

When a respondent chooses between two options, we call this “choice data”. When a respondent judges how much of an attribute in one option needs to be changed to make two options equal (or matched) we call this “judgment data”. Matching involves making judgments as opposed to choices. Time tradeoff data are judgments because, after bracketing through a series of choices, >