
# CAPTURING THE PATIENT VOICE IN VALUE ASSESSMENT: NEEDS FULFILLMENT

The Patient Access and Affordability Project (PAAP) Current Issue Report #1 on abandoning QALYs discussed how the imaginary worlds of the Institute for Clinical and Economic Review (ICER) lack scientific merit, and therefore its evidence reports – as imaginary constructs dogged by a failure to recognize the fundamental axioms of measurement theory – should be abandoned.

What should an alternative value assessment framework look like? We need a framework that actually accepts the standards of normal science, one that supplies health system decision makers with useful, replicable, actionable information. A key component of that framework is the patient voice as an accurate, quantifiable, understandable measure that reflects the benefit – or lack thereof – to the patient rather than the interest of clinicians or other non-patients.

## Direct and Indirect Benefits

Presumably, **the patient** is the recipient of the benefits of FDA approval and introduction to treatment practice. If we accept this proposition, then logically, our assessment of the benefits should relate directly to the patient.

Unfortunately, this proposition is far from being universally accepted. Rather, we have measures of response, patient reported outcomes (PROs) that reflect not the patient, but the interest of the physician in achieving operational targets. These are seen not only in generic multi-attribute systems such as the EQ-5D-3l/5L, but the multitude of disease-specific health-related quality of life (HRQoL) PROs. The focus of these instruments is typically on symptoms and functions. Benefit is inferred from the extent to which, within the symptoms of a disease that the developer considers appropriate, a therapy has some "positive" impact. However, **the patient may have a different perspective**. If we are concerned with the broad concept of quality of life (QoL), and if the presence of disease is a primary determinant of QoL, we need to capture the QOL attributes that are relevant to patients. We can't capture this critical information by inferring indirectly from what physicians believe is important. Physicians spend an average of 10 minutes with a patient during an office visit – clearly, that short window is hardly sufficient to probe beyond the more obvious manifestations of a disease. We must get QOL information from patients directly.

## Needs Fulfillment

If we are to understand how the QoL of patients is impacted by therapy options, then we need to dig deeper. More important, we need to consider the construct or attributes that characterize the QoL of patients in that specific disease state. The construct proposed by McKenna et al is "needs

fulfillment". [1] Rather than taking an operational view of therapy impact on symptoms and functional status within the various clinician-determined health dimensions considered relevant in that disease state, the focus is on patient value: the role of human needs in valuing life.

The needs approach to valuing therapy interventions is not new; it was proposed in the 1990s with antecedents in the Nottingham Health Profile of the late 1970s.[2] The needs model "*hypothesizes that the value of individuals' lives is dependent on the extent to which their human lives are fulfilled*." If a therapy meets few needs, then the value of that therapy – from the patient's perspective – is low. Clinical HRQoL measures would typically fail to capture or link to those attributes of **needs fulfillment** relevant to patients; functional scores for listed symptoms may "improve," but without benefit to the patient.

It is important to note that ICER's so-called "state of the art" value assessment framework relies on a generic US preference weighted multi-attribute "measure" of functional status within symptom dimensions. Benefit is inferred, not directly attributed, to these clinical parameters. In relying on the generic, this model necessarily disregards the specific. Put simply: ICER's model fundamentally disregards the patient voice.

## Protecting the Meme

The ICER model depends on protecting the meme, as patently absurd as it may be. A "meme" – an idea or a behavior that spreads within a culture from person to person – like this one can hit brick walls, or at least significant roadblocks, even if it has longitudinal high transmission fidelity. Unfortunately, there are many examples in the history of science where advocates have held on tenaciously to beliefs which were, by common consent, nuts. Perhaps the "meme pool" of technology assessment, to use in Dawkins'[3] turn of phrase, has to be subject to a Darwinian struggle for the current dominant meme supporting the construction of imaginary worlds to be overthrown. This struggle will commence in earnest when manufacturers and health system decision makers agree that the ICER model, and the construction of imaginary worlds, yield nothing of value.

As the meme dissolves, it will become painful evident that in taking the easy way out in creating imaginary world to support formulary decisions, manufacturers and health decision makers neglected the less-traveled road of actually developing platforms to evaluate and feedback results for credible cost-outcomes claims. Unfortunately, there are all too few patient centric instruments. They will take years to develop. And to date, there is little evidence that a reformation is in the offing with ISPOR reaffirming its commitment to the central placement of imaginary cost-outcomes claims as "approximate information" used for formulary decisions.[4]

## The Patient Voice

We now have over 20 years' experience in building needs-based response instruments that meet the required cardinal measurement standards, and that capture, in item selection and calibration a unidimensional measure of the direct impact of therapy choices on QoL, where QoL is the latent or attribute construct. This is achieved, as noted briefly in the previous report, on the rejection of classical test theory (CTT) in favor of Rasch Measurement Theory (RMT). If the item selection meets RMT standards, then they are confirmed as multidimensional with required cardinal properties. This is a critically important innovation, recognized now for some 30 years in health technology assessment, but for some 60 years in education and psychology.

Instrument development starts with agreement on a construct or theoretical latent framework that encompasses the attribute if interest – in this case, the QoL of the patient. The instrument

development is "bottom up," starting with in-depth qualitative interviews with patients to probe how the disease under review has affected the patient's life and how limitations of function impact the interviewees in that specific disease area. The outcome is the identification of a series of statements that capture the concerns for need fulfilment. Following traditional CTT assessments for face and content validity, a final item set is selected.

The key to the final item set selection is to subject the item set to RMT. The measure must create an index of patient value. If the items fit the RMT criteria, then the items are on the same measurement continuum. The overall score provides an index of the extent to which needs are met. If, for example, if the RMT calibrated scale yields 20 Yes/No item statements (e.g., I've lost interest in food), then the impact on the aggregate score of therapy options provides an index of response.

## Abandoning QALYs

A cardinal index of response, reflecting directly the patient's and target population's assessment of benefits is a far cry from the ICER imaginary value assessment framework. If we pursue the patient-centric, disease-specific approach to developing response instruments focused on QoL, then health decision makers will have to consider alternatives to the rejected cost-per-QALY value framework.

It has never been clear, at least in the US, why decision makers would be swayed by recommendations for downward-only price adjustments and access limitation constructed from models that are a black box to all but a few who have some training in these arcane imaginary constructs. Some decision-makers may take the ICER pronouncements at face value. CVS Pharmacy is a classic case in point – and cautionary tale – in its adoption of ICER cost-per-QALY imaginary constructs to bar products from its formulary.[5]

Others may simply see the ICER pronouncements as a bargaining chip, a means to accomplishing a business-driven goal rather than a patient-driven one.

If we are to subscribe to the standards of normal science – which formulary committees accept in drug development and the evaluation of competing clinical claims (or at least claims against placebo) – then we have to revisit formulary guidelines. Future Current Issue reports will consider potential strategies for evaluating response and outcome claims given two essential conditions: (i) any claim must be meet the standards of normal science, and (ii) any claim must provide feedback to formulary committees in a meaningful timeframe.

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