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The Opportunities and Challenges of Rethinking Our Approach to Value Assessment in Healthcare

As America's healthcare system continues to evolve, it is critical that our perception of care and its value to patients evolves with it. In the past, value assessments have marginalized patients' perspectives in favor of other, more easily quantifiable variables. Unfortunately, this approach to value assessment hasn't been able to capture individual health states or preferences because it fails to engage with the most important stakeholders: the individuals receiving the care.

Take, for example, the quality-adjusted life-year, also known as the QALY. Explained at the most basic level, the QALY is a measurement of how an intervention improves a patient's quality and quantity of life. The QALY aims to encapsulate the value of healthcare interventions in a single index number, where 1 equates to 1 year of perfect health and 0 is associated with death.

From the patient perspective, assessing the value and impact of care through a summary metric is akin to summarizing a 200-page novel in a single word. Although many experts acknowledge the limitations of the QALY metric, they often throw their hands up and assert that patient perspectives are just too difficult to quantify as a practicable metric.

But things are beginning to change.

This year, health economists and health services researchers rolled up their sleeves to offer alternative approaches to measure value as part of the Pharmaceutical Research and Manufacturers of America Foundation's 2019 Challenge Awards. The awards presented researchers with a single prompt:

"What are innovative, patient-centered approaches to contribute to healthcare value assessment that move beyond the inherent limitations of analyses based on the quality-adjusted life-year metric?"

Researchers responded with myriad novel, innovative, and practical approaches to value assessment that enhance or mitigate past the QALY and allow deep engagement with patients. Perhaps more importantly, the volume of substantive submissions undermined the idea that successfully incorporating the patient voice into healthcare assessments was too difficult.

Of all the approaches offered, 4 winning submissions were selected based on their innovative and pragmatic approaches to value assessment. Although each of the approaches differ in methodology and design, a common theme throughout is the realistic way in which they account for the perspectives of patients. In one selected model, for example, the authors propose to inform value assessment with learnings from patient-focused drug development (PFDD), a program instituted by the FDA to capture the experiences, perspectives, and needs of patients based on their unique symptoms and medical histories. Another approach argues for using patient stakeholder groups and existing patient registries to better incorporate patient perspectives. »

One of the submissions takes value assessment a step further, arguing that conventional approaches to value measurement that emphasize the average (mean) of healthcare outcomes are flawed. According to the authors, because patient care is about more than just average outcomes, we should incorporate the statistical variance and skewness of treatment outcome distributions to more effectively capture the wide spectrum of patient experiences.

Interestingly, many of the selected winners offer methodologies that aren't constructed from scratch. Instead, they expand on existing infrastructure, such as the PFDD, which is already purposed to capture patient perspectives on a medicine's effectiveness. Another winning submission discusses the advantages of applying multiple-criteria decision analysis—a long-standing decision-making tool used in other industries—given its ability to capture multiple dimensions of value that often go unmeasured.

But beyond just the winning approaches, many, if not most, of the submissions present realistic methods that expand the scope of value assessment, engage with patients, and innovate beyond the QALY.

It is clear that ongoing efforts such as the Challenge Awards that seek to move beyond the QALY are making meaningful headway in improving value assessments for patients. We should applaud the worthy goals of these efforts to fundamentally rethink what constitutes value. •

Eileen Cannon

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Here to Represent: Optimizing Representativeness and Enhancing Equity Through Patient-Engaged Healthcare Valuation

Lori Frank, PhD; and Thomas W. Concannon, PhD

Measuring value in healthcare is fundamental to delivering high-quality care and to quality improvement at the health-system, state, and federal levels. Assessing the value of new treatments, formalized through health technology assessment (HTA), also requires good measurement. Despite extensive documentation of drawbacks of the quality-adjusted life-year (QALY), the standard metric for HTA, alternatives have yet to gain wide acceptance.¹

The QALY invites the patient into valuation, but only as a research subject, not as a partner in defining relevant measure domains. This failure to represent patient views and priorities risks misspecifying value. QALYs derived from general public ratings of hypothetical health states, rather than from specific patient populations, raise both an ethical concern about representativeness and a methodologic concern about the accuracy of hypothetical-based “decision utilities.”^{2,3}

Recent reviews of value frameworks and HTA have raised the promise of multicriteria decision analysis (MCDA) as a path forward.⁴⁻⁶ Still missing, however, is a coherent, feasible strategy for incorporating active patient engagement into MCDA. Representation of relevant stakeholders is part of healthcare valuation, including QALY-based valuation, but implementation has fallen short. Collecting patient input is a recognized challenge to drawing links between clinical

evidence and health decision making by stakeholders.^{7,8} A survey of more than 30 HTA agencies found that very few use participatory strategies.⁹ In the United States, most value frameworks reference patient inclusion,^{10,11} but practical guidance on how to achieve inclusion is lacking.

Inadequate models of engagement lead to inadequate representation. For activities that involve valuation of healthcare, particularly in service of societal goals for equity in resource distribution, broad representation of affected groups is an ethical imperative.

Patient engagement and patient-centeredness are increasingly recognized as critical elements of healthcare quality. This was a core message of the Institute of Medicine's Quality Chasm report.¹² The Affordable Care Act and the 21st Century Cures Act further elevated the concept of patient-centeredness in healthcare and drug development.¹³⁻¹⁵ These cultural shifts offer a basis for overcoming the limitations of QALY-based value assessments by implementing equitable and representative inclusion of consumer views in defining and assessing healthcare value.

We propose a strategy for patient-engaged healthcare valuation to support patient and community engagement in valuation work, improving representativeness and ensuring that a wider range of affected stakeholders contribute to measuring value.

METHODS FOR A PATIENT-ENGAGED HEALTHCARE VALUATION STRATEGY

The methods below center on collection of patient-specific information to overcome the limitations of general population preference use and to incorporate patients' lived experience—the “personal” rather than the “vicarious”¹⁶—into the process through patient engagement and use of patient-engaged methods.

Criteria Elicitation and Selection: MCDA With Goal Attainment Scaling

This patient-engaged healthcare valuation strategy proposes employing MCDA methods using patient-informed criteria derived through goal attainment scaling (GAS).^{17,18} MCDA is a set of methods for decision analysis designed to elicit, collect, and use a range of decision criteria from multiple stakeholders in the service of healthcare decision making.^{19,20} Like all decision analysis, MCDA provides an ordered process to make decision making systematic rather than ad hoc or driven by overly narrow views.

The goals for GAS are jointly identified by patients and clinicians, and GAS is an effective measurement strategy when outcomes are heterogeneous, particularly with variable clinical presentation across patients. Obtaining criteria from patients based on individualized GAS-derived treatment goals answers the call for a wider range of dimensions than existing preference methods provide^{1,21} and ensures goals are patient important as well as clinically relevant.

IMPLEMENTING THE STRATEGY FOR PATIENT-ENGAGED HEALTHCARE VALUATION

1. Establish actively engaged large standing panels of patient stakeholders, with members trained in decision-analytic methods.

Select and train participants. Existing initiatives can serve as models.²²⁻²⁴ Patient panels could facilitate MCDA exercises and take the lead on criteria identification through GAS.

Identify “engagement liaisons” to connect with relevant patient communities and to facilitate training activities. Engagement liaisons can work with multiple communities in a “hub-and-spoke” model. The liaison “hubs” can be networked in a learning community, and patient “spokes” can use existing patient online forums and establish new ones to achieve active connections.

2. Use direct-to-patient crowdsourcing to enhance patient engagement in valuation work.

Online surveys are already used to collect preference and other patient data.²⁵ The opportunity now is to expand representativeness through online engagement with heterogeneous sets of patients. This strategy shifts the focus to an active bidirectional engagement model rather than passive 1-way data collection from subjects.

Crowdsourcing overcomes some logistical challenges involved in the collection of health states from the general population and allows

for both generic and disease-specific health state collection.²⁶ Goals elicited through panels in step 1 ensure active patient goal generation and can be adapted for larger-scale endorsement in step 2. In this way, elements important to specific patient communities can be incorporated into larger-scale data collection for verification and to inform goal prioritization and weighting.

3. Adopt patient-engaged MCDA in HTA.

For valuing elements of healthcare, the steps above support representativeness of a decision analytic process and can enhance inclusion of diverse perspectives relevant for equitable distribution of resources and, ideally, health gains. By making patient values central, patient-engaged MCDA would enhance both equity and representativeness.^{4,21,27}

GAS can aid with MCDA steps like construction of value trees, the hierarchical structure or schematic representation of evaluation criteria and attributes, and scoring, the criteria-based rating of preferences for options.^{28,29} GAS can simplify collection of patient input while preserving the emphasis on patient-important outcomes. Both GAS and MCDA provide a structure for the interaction of patients with others, an important element to help avoid the “disappointment” researchers can experience with some forms of patient participation in HTA.³⁰ Web-enabled software has advanced feasibility.^{31,32}

CONCLUSIONS

The strategy presented here accomplishes multiple goals: engagement with patients whose training improves the efficiency of valuation exercises, availability of large samples when needed, and wider patient representativeness in the results. Available technologies for large-scale direct-from-patient data collection can advance the ethical and practical benefits of increasing stakeholder involvement in healthcare valuation. With wider adoption of methods like MCDA to expand criteria included in valuation exercises, connection to patient criteria is more urgent, making GAS an important element for incorporating first-hand experience. Developments in patient engagement have set the stage for technology-aided communication with patient communities and can substantially improve the representativeness of patients in valuation work beyond current tokenistic levels. •

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