



# The Importance of Collaboration in Pursuit of Patient-Centered Value Assessment

Mark T. Linthicum<sup>1</sup> · Susan dosReis<sup>2</sup> · Julia F. Slejko<sup>2</sup> · T. Joseph Mattingly II<sup>2</sup> · Jennifer L. Bright<sup>1</sup> 

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## 1 Introduction

Economic theory suggests that patients as the ultimate consumers of care are an essential component of any assessment of value in healthcare [1, 2]. Economic analysis aims to support efficient resource allocation by shedding light on the value of available alternatives, but capturing patient-centered value remains the focus of much theoretical debate and methodological uncertainty. Major advances not only in methods for patient-centered value analyses but also in building a patient-centered research environment are needed. We are making early progress, but this can be accelerated through coordinated and collaborative efforts.

In the healthcare context, “value” can be defined in many ways, but economists generally view value as the relative benefits, costs, and risks of competing alternatives. Applying this definition, healthcare value assessment is the systematic evaluation of the relative benefits and costs of medical technologies and healthcare interventions to guide budget-constrained resource allocation decisions [3]. Value assessment, also referred to as health technology assessment, draws upon methods and evidence from medicine, epidemiology, and economics for health-state utility measurement, health economic modeling, and decision analysis to evaluate the value of healthcare interventions in terms of their benefits relative to their costs.

Much debate surrounds what exactly should be included in estimating benefits and costs, however, and the growing use of value assessments in coverage and reimbursement decisions around the world has been accompanied by widespread calls for better representation of patients’

experiences. A common criticism of cost-effectiveness analyses, for example, is that the use of quality-adjusted life-years to capture the benefits of therapies does not adequately capture societal benefits or the relative importance of various outcomes to patients [4]. Some health technology assessment agencies and value assessment organizations (e.g., the Institute for Clinical and Economic Review in the USA and the National Institute for Health and Care Excellence in the United Kingdom) include qualitative data on patient experience as a supplement to quantitative value assessments, but patient-centered quantitative assessment remains an aspiration [5–9].

Several specific considerations must be addressed for quantitative value assessments to become more patient-centered, including the need to:

1. Clarify how to operationalize the role of patients, caregivers, and other stakeholders at all stages of research, analysis, and decision making.
2. Develop a measurable understanding of the clinical and non-clinical outcomes that are important to patients.
3. Expand data collection and evidence development approaches on how interventions affect outcomes of importance to patients. Incorporating additional patient outcomes, for example, days of work missed, requires sufficient supporting evidence to parameterize models and differentiate across interventions.
4. Improve methods for quantifying outcomes that are not easily translated into costs or health-state utilities. For example, quantifying preference for improvement in social activities enables the estimation of the threshold for risk that outweigh gains in benefits [10].
5. Advance methods for translating patient-driven data and evidence into modeling and value assessment and for applying these measures to meet various decision-makers’ needs.

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✉ Jennifer L. Bright  
jennifer.bright@thevalueinitiative.org

<sup>1</sup> The Innovation and Value Initiative, Alexandria, VA, USA

<sup>2</sup> Patient-Driven Values in Healthcare Evaluation (PAVE) Center at the University of Maryland School of Pharmacy, Baltimore, MD, USA

Addressing the methodological issues described above and building a relevant evidence base requires considerable time and resources, including focused and coordinated efforts across fields, disciplines, and industries. A commitment to patient-centered research and decision making, which means including patients throughout the research process, is essential to addressing questions about what is valuable for patients.

## 2 New Collaborations in Patient-Centered Value Assessment

Many organizations and individual researchers are working to identify scientifically valid methods to improve value assessment. This change will not happen all at once, and we see collaboration across stakeholder groups as central to meaningful progress towards patient-centered value assessment.

Despite the significant challenges in shifting the value assessment research practices to include patients, existing collaborations provide examples of how partnerships may help to make meaningful progress toward more patient-centered value assessment. Our organizations, the Patient-Driven Values in Healthcare Evaluation (PAVE) Center at the University of Maryland School of Pharmacy and the Innovation and Value Initiative (IVI), were founded on the premise that patient perspectives need to be accurately incorporated when defining value, but our specific approaches differ. By leveraging complementary skills and expertise, we are able to improve the scientific methods needed for patient-centered value assessment.

A partnership between researchers at the University of Maryland and the National Health Council, The PAVE Center develops methods for patient-driven value assessment. PAVE Center researchers collaborated with patient stakeholder partners to generate a core set of value elements designed to capture the relative importance of the attributes of healthcare interventions and associated outcomes to patients. These elements complement outcome-focused standard sets developed by other organizations [11, 12], focusing on quantifying patient preferences regarding treatments' short/long-term effects, costs, access to treatment, and the impact of treatment on life and social well-being, with a focus on quantifying patient preferences. With patient stakeholder engagement, these value elements can be tailored to specific medical conditions and quantified<sup>1</sup> to

support a patient-informed reference case in a cost-effectiveness analysis [13, 14].

Building on this core set of value elements, the PAVE Center team conducts cutting-edge research to reflect meaningful patient experiences into economic evaluations [15]. As a first step, PAVE works with patients to identify what is most important to their daily life experiences; [16, 17] current research focuses on chronic obstructive pulmonary disease and patients and caregivers impacted by severe food allergies. This research will generate quantifiable measures of patients' benefit-risk trade-offs that can be incorporated into an economic evaluation.

Translation of new methods and evidence into the practice of patient-centered value assessment requires ongoing testing and refinement. IVI's goal as a non-profit research organization is to create an environment that facilitates translational research in patient-centered value assessment and accelerates the development of both methods and evidence. As part of the Open-Source Value Project, IVI develops disease-specific model prototypes to test evolving methods, identify gaps in existing approaches, and engage different stakeholders in "road-testing" and improving value assessment [18].

Development of these models provides many opportunities to test and improve methods. For example, IVI partners with members of patient communities as a first step in model development, and input from these partners and pre-development research on patient experiences and value inform model development from the start. IVI's models are open source and designed to be fully customizable, making it possible to modify analyses for specific decision contexts and model patient heterogeneity [19]. Preference-weighted evaluation such as a multi-criteria decision analysis is supported in addition to a cost-effectiveness analysis, which makes it possible to include patient-identified treatment attributes that are difficult to include in a cost effectiveness analysis (such as time since US Food and Drug Administration approval) and to adjust value based on the relative importance of attributes to patients [14, 20]. Importantly, IVI's models are developed, tested, and improved with the input of diverse experts, from economists to providers and patient communities to insurers and employers. In addition, IVI's Patient Advisory Council provides ongoing feedback and insights into the organization on how it can fully integrate patient perspectives into its organizational and research process.

As IVI began early planning for the development of a new model focused on major depression, the opportunity for collaboration between IVI and PAVE quickly became clear. PAVE researchers' expertise in the estimation of patient preferences on value is providing the foundation for early-stage model development. For example, PAVE researchers are working with patients to identify key determinants of

<sup>1</sup> By tailoring the elements to specific medical conditions, this enables measurement of preferences for the value elements, their relative importance, and the weighting of benefits/risks with outcomes in decision making.

value from their perspectives. Moreover, IVI's multi-stakeholder advisory group, which includes patient organizations, can offer first-line input into the definition of attributes used in PAVE's inquiry with patients. This research will contribute to the design of the economic model, making it possible to explore additional methodological innovations of interest to both PAVE and IVI researchers. This partnership benefits all involved: we build our patient experience evidence base through quantifying value elements identified by PAVE, while IVI model development extends methods in parameter uncertainty and preferences for value estimates. Collaborative efforts such as this partnership not only help our organizations pursue their own objectives, but also accelerates the research process overall.

The partnership between PAVE and IVI moves the needle on important issues, yet this is only the beginning. Patient-centered understandings of value begin with basic evidence generation—trials that identify the outcomes that matter most to patients and then generate evidence on these outcomes and related outcomes such as adherence, for example. Similarly, ongoing evidence generation is needed in real-world practice. This requires not only collaboration across disciplines and organizations, but also the improvement of methods for collection and the use of observational data in decision making.

In the economics-based value assessment space in which IVI and PAVE are focused, countless opportunities exist for fruitful collaboration, between researchers exploring new methods, health systems with access to real-world data, and engaged patient communities interested in sharing data, to name a few. Potential partners must not only be open to collaboration but be prepared to actively pursue it, especially where institutional barriers (concerns about sharing proprietary data, for example) or distrust across parties may exist.

### 3 Conclusions

The raw materials exist for patient-centered value assessment, but additional translational research is needed to advance societal benefit and influence healthcare decision making. A focused and coordinated effort in the fields of health economics and outcomes research is necessary to translate these concepts into practice. Improving clinical and health system research, especially in the fields of economics and epidemiology, to better capture patient-level experiences serves multiple key purposes: understanding what is important to patients, identifying unmet needs, and generating value estimates that more accurately capture the value of therapies and treatment strategies are just a few examples. Patient-centered value assessment is both scientifically feasible and relevant to real-world decisions across the range of stakeholders. Organizations like IVI and PAVE represent

a model collaboration; with our combined expertise and resources, we can accelerate the research and implementation of patient-informed value assessment.

#### Declarations

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