

Value Assessment That Puts Patients at the Forefront

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- The authors take an in-depth look at living with a life-changing disease, and how the treatments of those diseases focus too narrowly on clinical outcomes.
- More needs to be done to hear the patient voice to determine what matters most to patients.
- Several new patient-centered approaches to value assessment that will help reduce the current overabundance of low-value care are highlighted.

Healthcare decisions regarding treatment options and plan coverage need to be well-informed—by both clinical endpoints and nonclinical patient experience data—as these decisions have a wide-ranging impact not only on patients and their families, but the entirety of our healthcare system. Unfortunately, there are large gaps in evidence of the effectiveness and value of healthcare that is delivered to patients around the country. This is in part because experiences and outcomes that matter to patients are often excluded from data collection processes intended to help inform the value of health services and interventions. This disproportionate focus on clinical outcomes in health technology assessments can lead to highly problematic and unintentional consequences.

Assessments meant to determine the value of such treatments could be missing crucial elements that define what it is like to live with a life-changing disease. For example, in the cancer treatment community, the measurement of outcomes such as overall survival, time spent in the hospital, and other clinical endpoints are favored over those that comprehensively measure patient quality of life—if quality of life, or patient experience measures are even captured adequately or at all. As a result, assessments meant to determine the value of such treatments could be missing crucial elements that define what it is like to live with a life-changing disease.

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Further, traditional assessments might be using measures that are not well understood by patients. A recent cross-sectional study of cancer patients and survivors found limited awareness and minimal understanding among the patient community about how quality-adjusted life years (QALY), a common measure

used in health technology assessments, was used in making healthcare decisions. Only one-quarter of patients surveyed by researchers believed that the QALY was a good way to measure value in healthcare and many expressed concerns about how it would be used by payers, policy makers, and other decision makers in determining access to treatment.¹

Hearing the Patient's Voice

Encouragingly, policy makers and the value assessment community are beginning to recognize the necessity of the patient voice and why it is important to measure outcomes that matter to patients (ie, patient-centered outcomes) and ensure they are included in determinations of value. In June 2020, the US Food and Drug Administration (FDA) announced a pilot program to communicate patient-reported outcomes from cancer clinical trials to complement existing labeling and patient information about cancer treatments.² This initiative, [Project Patient Voice](#), is an important first step towards stimulating a broader conversation about value within the chronic disease community.³ Other projects such as the Cancer Support Community's [VOICE \(Valued Outcomes in the Cancer Experience\)](#) tool are designed to measure patient priorities and understand discrepancies between what matters most to patients and what patients believe they can control.⁴

On the academic side, researchers, with the support of the PhRMA Foundation,

are developing and advancing methods of value assessment in healthcare and are beginning to broaden the view of value across the system to support the identification and removal of low-value care. From this research, exciting new approaches are emerging to generate evidence that informs value-based

decision making and tackles different challenges in healthcare delivery.

Including Novel Value Elements in Economic Evaluations

Conventional value assessment models often fail to successfully engage patients and fully capture their unique perspectives during healthcare value discussions. To address the shortcomings of traditional value assessment models, the [Patient-Driven Values in Healthcare Evaluation \(PAVE\)](#), a partnership between the University of Maryland's School of Pharmacy and the National Health Council, has been working to develop more patient-centered approaches to value assessment by engaging directly with patients to understand and quantify the value elements that matter most to them.^{5,6} Their most recent study engaged patient stakeholders from diverse medical backgrounds in an iterative process to develop a core set of value elements that can be incorporated into economic evaluations of healthcare interventions. Notably, approximately 75% of the value elements researchers identified were generally not used in existing value frameworks.⁷

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PAVE's patient-driven approach to value assessment can be seen through their recent partnership with the COPD Foundation, a leading patient-focused organization that provides a voice to the concerns and needs of patients with chronic obstructive pulmonary disease (COPD). PAVE researchers are working hand-in-hand to identify outcomes important to patients diagnosed with COPD and incorporate these novel value elements into economic evaluations in a way that can be quantified.⁸ PAVE is also partnering with the Asthma and Allergy Foundation to identify outcomes that matter to families of children with severe allergies and is working to ensure that patient advocacy organizations are aware of the tools being developed to empower patients to amplify their voices in value assessment discussions.

Moving Past Conventional Cost-Effectiveness Methods

The [Center for Pharmaceutical Value \(pValue\)](#), established at the University of Colorado's Anschutz Medical Campus, aims to apply and test novel methods for value assessment that incorporate multicriteria decision analysis (MCDA) to inform coverage decisions in the United States.

MCDA, which has been used in other sectors outside of healthcare for many years, offers a structured and transparent method to account for all criteria important to a decision. With its ability to clearly view evidence involved in the decision process, MCDA can help identify and address evidence gaps, which may be particularly relevant in the rare and ultra-rare disease space where researchers often encounter sparse evidence.⁹

pValue's research aims include the development of a patient-centered outcomes inventory table to bring structure and

importance to patient-centered outcomes not incorporated in traditional value assessment using rare disease as the initial case examples. pValue also aims to assess stakeholder perception of value based through multistakeholder engagement efforts. The flexibility associated with using MCDA alongside traditional methods for value assessment, such as the ability to strategically select and weigh inputs that matter most to stakeholders, will support those making coverage decisions to do so based on evidence relevant to their respective member populations.

Enhancing Cost-Effectiveness Methods

Recognizing that common value tools may not fully capture patient and societal well-being, the [Center for Enhanced Value Assessment \(CEVA\)](#) at Tufts Medical Center expands upon traditional measures of value by exploring the integration of nontraditional elements in value assessment. These include patient-centered factors such as the value of hope, family spillovers, and medication adherence. Other factors such as the risks and uncertainties associated with treatment, the financial burden on patients, the impact of the treatment on worker productivity, scientific spillovers, and equity will also be explored.

CEVA researchers plan to engage multiple stakeholders—patients, health insurers, and therapeutic area leaders—to characterize these nonstandard elements and present this information to stakeholders, alongside standard value elements and cost-effectiveness information, in the form of case studies.¹⁰ CEVA's output from these case studies could inform coverage and reimbursement decisions by payers that better align and meet the needs of their respective member populations.

Reducing Low-Value Care

A substantial amount of the US healthcare budget is spent on services and procedures deemed “low value” and medically unnecessary. Eliminating spending on care that offers little-to-no value to patients would not only facilitate the shift towards a value-driven health system but would also result in immediate and substantial system savings to facilitate the reallocation of resources towards services that provide high value to patients. As such, it is important to focus on how much and how well we spend budgets across the entire spectrum of care delivery.

This is the philosophy advocated by the [Research Consortium for Healthcare Value Assessment](#), or Value Consortium for short, which has brought together healthcare researchers to collaborate, share findings, and develop research strategies that would address inefficiencies in healthcare. The Value Consortium focuses on identifying low-value clinical services (ie, care that offers little-to-no value to patients) and tracking the use of and spending on such services. Their objective is to arm employers, providers, health systems, and state agencies with data and tools to understand how much of their spending is allocated towards low-value care and how wasteful spending can be reduced.¹¹ The absence of readily available data for payers, such as state agencies or employers, to understand their spending on low-value care is a challenge the Value Consortium aims to tackle. Value Consortium researchers have conducted major analyses of private payer claims data to identify the prevalence and utilization of 20 low-value services and have also developed a publicly available [online tool](#) that stakeholders can use to

monitor and track low-value care services in their respective states.^{12,13} The Value Consortium has also announced a new project that will begin to explore how healthcare resources can be used more wisely in the aftermath of COVID-19 in order to help hospitals and other providers cut waste and support a more robust, evidence-based understanding of value in healthcare.¹⁴

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Looking Ahead

The movement within the scientific research communities and the federal government to include the patient voice in healthcare decision making is gaining momentum. These initiatives are innovative approaches that move beyond the status quo of healthcare value measurement and assessment. The shift towards a value-driven health system requires transparent approaches that keep the patient front and center and will ensure payers make informed coverage decisions that reflect the unique characteristics of the members they represent. In doing so, these decisions will have a more sustained, positive impact on our healthcare system's budgetary strains. Beyond their innovative efforts, these research groups are forging a path to the end game in healthcare: a healthy patient population that receives the highest quality of care and delivers the most valuable outcomes. •

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