Driving Progress in U.S. Value Assessment

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Authors

Emily Ortman

Kimberly Westrich, MA

Lisabeth Buelt, MPH

Author Affiliations

Emily Ortman is the Head of Communications at the PhRMA Foundation. Kimberly Westrich is the Director, Value & Access Strategy at AmerisourceBergen/Xcenda, and Lisabeth Buelt is the Manager, Value & Access Strategy at AmerisourceBergen/Xcenda.

About the PhRMA Foundation

The PhRMA Foundation fosters biopharmaceutical innovation and value-driven health care by investing in the frontiers of research. The Foundation catalyzes the careers of promising researchers through competitive, peer-reviewed grants and fellowships in the fields of drug delivery, drug discovery, translational medicine, and value assessment and health outcomes research. Since its founding in 1965, the Foundation has awarded more than $110 million to over 2,700 researchers from diverse backgrounds at more than 300 institutions. To learn more, please visit www.phrmafoundation.org.

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Executive Summary

To curb rising health care spending in the United States while also optimizing the quality of care, stakeholders are increasingly implementing value assessment tools to determine the relative benefits and costs of health care interventions. Patients and health care providers are using these tools to guide shared decision-making while health care payers, such as insurance companies, are using them to make decisions about what medicines or health care services they will cover.

This analysis reviews the value assessment landscape in the U.S. and examines current assessment tools to better understand their respective structures, methodological approaches, and assessment processes. It also outlines several key challenges that may hinder the robust application of value assessment within the complex, multipayer U.S. health system.

Addressing these gaps requires cultivating cutting-edge research in the field of value assessment. The PhRMA Foundation is a nearly 60-year-old nonprofit that fosters biopharmaceutical innovation and value-driven health care by investing in the frontiers of research. Through competitive, peer-reviewed grants and fellowships, the Foundation supports investigator-driven research focused on the development of advanced value assessment methodologies that are rigorous and transparent and address the needs of all health care stakeholders. This paper highlights how Foundation-funded researchers are working toward solutions that will advance the field of value assessment.

The PhRMA Foundation
is investing in new research to advance the field of value assessment.

Identifying Current Challenges

Insufficient consideration of health equity

Shortcomings in existing value assessment methods

Reliance on narrow definition of value misaligned with patient needs

Supporting Solutions-Focused Research

Striving to improve health equity

Advancing rigorous and transparent value assessment methods

Broadening the definition of value and prioritizing patient focus and engagement

KEY TAKEAWAYS

The landscape for value assessment in the U.S. is maturing, but opportunities exist to improve value assessment methods and tools.

Gaps in current value assessment methods may lead to health care decisions based on incomplete assessments that are misaligned with patient needs.

The PhRMA Foundation is cultivating investigator-led research that advances the field of value assessment.

Fully addressing existing challenges in the field of U.S. value assessment will require multistakeholder collaboration and additional sustainable funding streams.

Background

To improve the efficiency and quality of health care delivery in the United States, policymakers and other health care stakeholders are implementing changes to transition to a more value-based health care system. This means incentivizing the quality of care as opposed to the quantity of services provided and putting a greater focus on patient health outcomes. To help facilitate this shift, many stakeholders are looking to value assessment to promote the delivery of high-value care.

Value assessment focuses on evaluating health care interventions — such as pharmaceuticals, medical devices, or medical procedures — to define and quantify their value.[1] Methods for value assessment vary in sophistication and scope. For example, a value assessment may rely on comparative effectiveness research (CER), which compares the benefits and risks of an intervention to one or more other interventions to determine its relative value. A value assessment may also include cost-effectiveness analysis (CEA), which combines CER with an economic assessment to determine the relative value of a health care intervention based on both its benefits and its *cost*.

Health technology assessment (HTA) is a formal, systematic form of value assessment. Government-designated HTA agencies are common in Europe and some other countries, where their findings are used to decide whether to provide access to new health interventions. In recent years, the U.S. has seen steady growth in the number of organizations conducting value assessments. National organizations such as the Institute for Clinical and Economic Review (ICER), the Innovation and Value Initiative (IVI), and the National Comprehensive Cancer Network (NCCN) have introduced value assessment frameworks and tools to guide health care decision-makers in evaluating the relative benefits and costs of health care interventions, primarily pharmaceuticals. These tools are intended for a broad constituency of end users and serve different purposes, such as guiding shared decision-making between patients and their providers or serving as a resource for health care payers (such as insurance companies) as they make decisions about what medicines or health care services they will cover.

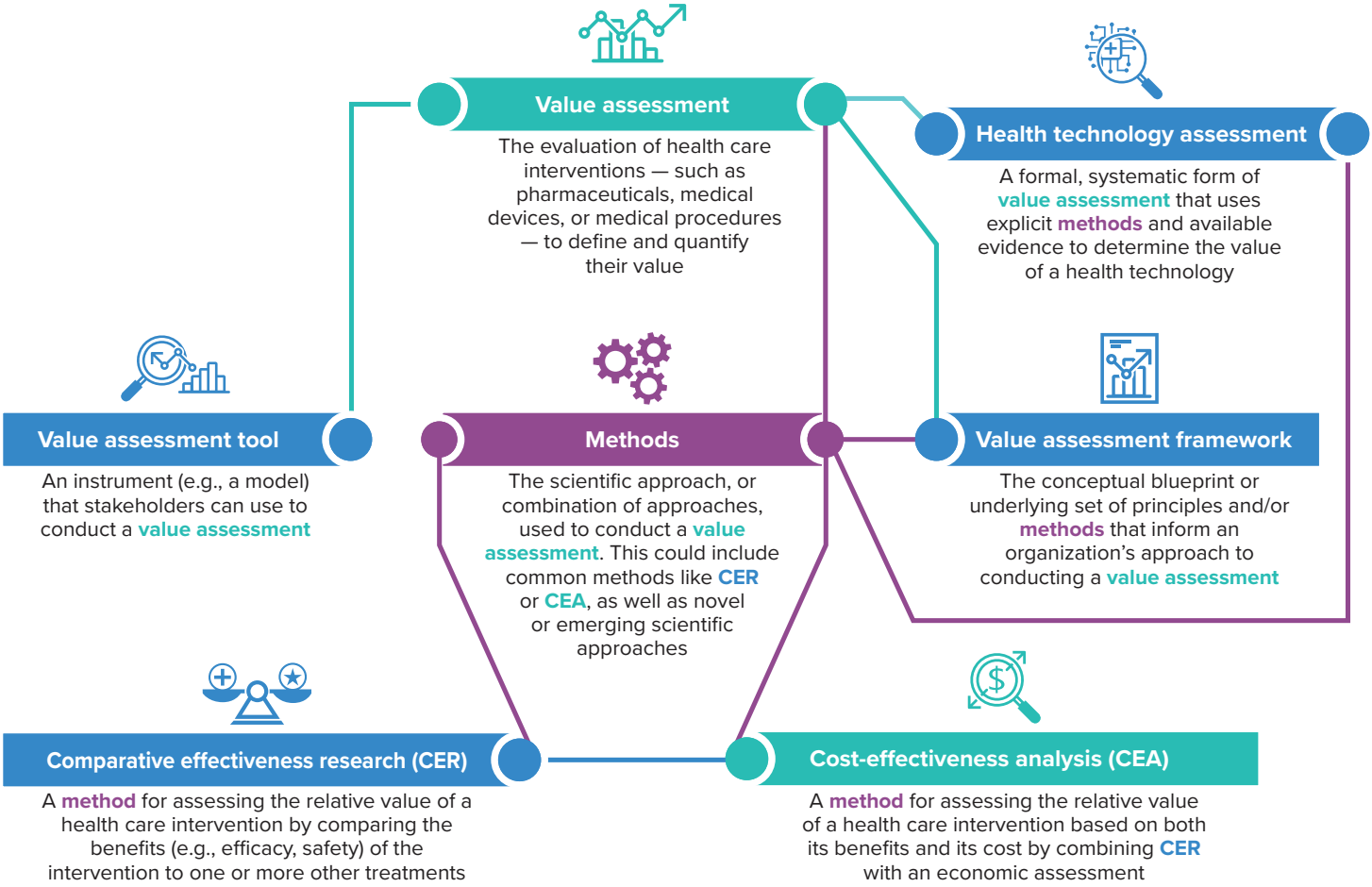
The field of value assessment in the U.S. is dynamic and evolving, and the use of value assessment is gaining traction with health care decision-makers and policymakers. A recent analysis found that 79% of surveyed health care payers reported that ICER recommendations influenced their decision-making in 2022, compared with only 49% in 2016.[2,3] In 2021, the University of Southern California (USC) Schaeffer Center for Health Policy & Economics and the Aspen Institute convened an advisory panel that recommended bolstering HTA activities and establishing a publicly funded, advisory-only HTA body in the U.S.[4]

Additionally, in August 2022, the Inflation Reduction Act was signed into law, ushering in numerous health care reforms, including a provision that will allow Medicare to “negotiate” the price of select prescription drugs beginning in 2026.[5] Although it is still uncertain how this provision will be implemented, the law stipulates that the Secretary of Health & Human Services should consider several factors to inform these negotiations, including the comparative effectiveness and cost of therapeutic alternatives, potentially paving the way for the U.S. government to formally incorporate value assessment in its decision-making. Policymakers responsible for implementing the law have noted that the policy creates the potential for a national value framework.[6]

As health care stakeholders increasingly use value assessment tools to inform their decision-making, it is crucial that these tools rely on validated, comprehensive, and patient-centered methods. Reliance on narrow or underdeveloped methodologies could create unintended barriers to patient access to important treatments and result in health care decisions that are misaligned with patient needs. While value assessment presents an opportunity to promote value-driven health care, current methodological gaps and uncertainties in the field may prevent its optimal application within the U.S. health system. To address these gaps and cultivate new research in the field of value assessment, the PhRMA Foundation is investing in the development of advanced value assessment methodologies that are rigorous and transparent and address the needs of all health care stakeholders — especially patients.

This analysis reviews the value assessment landscape in the U.S. and examines current assessment tools to better understand their respective structures, methodological approaches, and assessment processes. It also highlights key challenges related to assessing value within a complex, multipayer health system and explore how the PhRMA Foundation is investing in transformational research to advance methodological approaches, improve the inclusion of patient-focused value elements, and integrate health equity considerations in the field of U.S. value assessment.

Figure 1: Key value assessment terms



Examining U.S. Value Assessment Tools

Numerous U.S. organizations have developed value assessment frameworks or tools, each with a unique purpose, methodological approach, and intended audience.[7]

Some organizations, including health care provider groups or clinical practice guideline bodies, have created value assessment tools to help inform shared decision-making between patients and their care teams, including:

- American Society of Clinical Oncology's (ASCO) Value Framework[8]
- NCCN's Evidence Blocks (EBs)[9]
- American College of Cardiology and the American Heart Association's (ACC/AHA) recommendations for implementing value assessment in ACC/AHA clinical practice guidelines[10]

Other organizations have developed value assessment tools to inform policy discussions and health care coverage decisions, including:

- ICER's evidence reports[11]
- IVI's Open-Source Value Project (OSVP) models[12]
- Drug Pricing Lab's Drug Abacus (originally developed by researchers at Memorial Sloan Kettering Cancer Center)[13]

Several organizations have released resources on best practices to promote evidence-based methods and improve the patient focus of value tools, including:

- International Society for Pharmacoeconomics and Outcomes Research (ISPOR) Special Task Force on U.S. Value Assessment Frameworks[14]
- National Health Council's Patient-Centered Value Model Rubric[15]
- Avalere/FasterCures' Patient-Perspective Value Framework[16]

To develop new tools and methods for assessing value, the PhRMA Foundation provided grants to support the creation of four academic Centers of Excellence in Value Assessment:

- Center for Enhanced Value Assessment (CEVA)
- Center for Pharmaceutical Value (pValue)
- Patient-Driven Values in Healthcare Evaluation (PAVE)
- Research Consortium for Health Care Value Assessment

The wide-ranging, multidisciplinary activity of all these organizations has helped define and advance the field of value assessment in the U.S. However, only three organizations are actively conducting organizational-led value assessments to guide stakeholder decision-making:

- ICER
- IVI
- NCCN

To better understand the strengths and limitations associated with the value assessment tools from these organizations, we evaluated them across three broad categories: structure, methodological approach, and assessment process (**Table 1**).

Table 1. Overview of U.S. value assessment tools

	ICER Evidence Reports	IVI Open-Source Value Project Models	NCCN Evidence Blocks (EBs)
STRUCTURE			
Target audiences	Payers and policymakers	Patients, providers, policymakers, manufacturers, and payers	Providers and patients
Services assessed	Primarily pharmaceutical treatments, some health care services	Primarily pharmaceutical treatments	Pharmaceutical treatments
Conditions assessed	Any condition	Any condition	Oncology
Assessment output	Comparative cost-effectiveness estimates / Health-benefit price benchmark / Budget impact estimates	Customizable comparative cost-effectiveness estimates / Customizable assessment of overall value based on MCDA	EB scores for five domains: efficacy, safety, quality of evidence, consistency of evidence, affordability
METHODOLOGICAL APPROACH			
Approach overview	Comparative clinical effectiveness analysis / CEA / Budget impact assessment	CEA and MCDA	Average of NCCN expert panel scores for a drug or regimen using a five-point scale for each value domain
Transparency/ replicability	Model analysis plan is publicly available, but the model itself is not / Model transparency program allows some manufacturers temporary access	All model code is open-source and publicly available, as is R statistical package and detailed documentation	Individual panel member scores are unknown and are not reproducible
Types of evidence	RCTs, NMAs / May consider additional types including observational studies, RWE, manufacturer-submitted evidence	RCTs, RWE	Expert panel members' clinical expertise and knowledge of the evidence base underlying the NCCN Clinical Practice Guidelines (e.g., RCTs)
Measure(s) of efficacy	No standard measure: varies by assessed condition	No standard measure: varies by assessed condition	Treatment's ability to prolong life, arrest disease progression, or reduce symptoms
Measure(s) of patient-centered/ indirect benefits	QoL included in CEA / Productivity included quantitatively in scenario analyses / Varies by report, but has included acuity of need, magnitude of lifetime impact, patients' ability to achieve major life goals, caregiver and family QoL, complexity of regimen, and reducing health inequities	Varies by model, but has included reduction in patients' earnings, route of administration, productivity, variable patient response, variable time horizon, value to the currently healthy, value of hope	None
Measure(s) of cost	Net cost of drug to health system payer / Medical cost offsets / Medical costs (data permitting)	Net cost of drug to health system payer / Medical costs	Cost of the drug to the health system payer / Medical costs
ASSESSMENT PROCESS			
Number of assessments*	65 completed reviews** / 4 in-process reviews**	2 completed models / 1 in-process model	EBs included in 61 NCCN Guidelines
Public comment	Opportunity for public comment on draft scoping / document and draft evidence report / Public meeting allows time-limited invited comments	Opportunity for public comment on model scope, model protocol, and following each model release	No public comment period in the development of EBs / Stakeholders can submit data and request a change after an EB is published
Patient engagement	Includes patient stakeholders in its assessment process	Includes patient stakeholders in its model planning and development processes	None

* Number of assessments as of December 2022. ** Topic reviews counted since implementation of ICER Value Assessment Framework.
CEA – cost-effectiveness analysis; EB – evidence block; ICER – Institute for Clinical and Economic Review; IVI – Innovation and Value Initiative; MCDA – multi-criteria decision analysis; NCCN – National Comprehensive Cancer Network; NMA – network meta-analysis; QoL – quality of life; RCT – randomized clinical trial; RWE – real-world evidence.

Structure

This report evaluates the structure of each assessment tool, including scope of topics, target audience, and assessment output.

ICER's Evidence Reports

In 2015, ICER, a nonprofit research organization, released the first iteration of its value assessment framework, which provides the methodological foundation for its evidence reports. ICER's reports summarize the evidence on assessed interventions' clinical comparative effectiveness and include CEA calculations that reflect the degree of improvement expected in long-term patient outcomes. They also include value determinations based on organizationally defined willingness-to-pay thresholds (an estimate of what a health care consumer might be willing to pay for the health benefit).

Health care payers and policymakers are the primary audiences for ICER's evidence reports; however, patient groups, providers, and researchers are all secondary audiences. ICER's evidence reports take a "population" level perspective and provide analyses that support population-level (vs. patient-level) decision-making, including pricing and coverage determinations. Although ICER occasionally reviews non-pharmaceutical treatments, most of its reports focus on medicines. ICER assessments can examine any condition (i.e., ICER does not exclusively review treatments for a specific disease area like oncology).

ICER evidence reports include comparative cost-effectiveness estimates that approximate the incremental cost for an additional quality-adjusted life year (QALY) relative to a comparator. A QALY is a unit of measurement for how well a treatment lengthens or improves patients' lives, with one QALY equal to one year of life in perfect health. Notably, health care stakeholders including bioethicists, patient advocates, and policymakers, have voiced ethical concerns over the use of the QALY in value assessment, asserting that the QALY calculation undervalues treatments and services for patients with chronic illnesses or disabilities who may never achieve perfect health.[17,18]

ICER's reports produce health-benefit price benchmarks, which represent the price needed for a treatment to meet ICER's designated cost-per-QALY threshold. ICER also assesses the potential budget impact associated with assessed interventions and provides policy recommendations.

IVI's Open-Source Value Project Models

IVI is a nonprofit research organization founded in 2016. IVI's OSVP models are intended for a broad audience of policymakers, health plans, providers, life science firms, and patients. Historically, IVI's models have focused solely on pharmaceutical interventions, but IVI recently announced its intention to incorporate non-pharmaceutical interventions in its review of major depressive disorder.[19] Like ICER, IVI assessments can examine any condition.

In contrast to ICER, IVI does not publish treatment-specific value determinations. IVI's models allow users to conduct value assessments using both CEA and multi-criteria decision analysis (MCDA). MCDA allows end users to weigh various value elements so that the resultant value determination reflects their individual preferences and priorities. Similar to ICER's methodology, IVI's CEA method calculates estimates of cost-per-QALY gained; however, OSVP models allow users to choose their willingness-to-pay thresholds and customize their value determination results using modifiable inputs for measures related to a treatment's benefits and cost.

NCCN's Evidence Blocks

NCCN is a nonprofit alliance of 32 leading cancer centers. NCCN's EBs are designed to help patients and their care team select oncologic therapies based on standardized metrics related to a treatment regimen's efficacy, safety, cost, and underlying evidence. EBs are incorporated into NCCN's guidelines and focus solely on oncologic pharmaceutical treatments. The primary audience for NCCN's EBs is guideline users, including health care providers and patients; however, payers also use NCCN EBs to inform formulary decisions.[20]

NCCN's EBs employ a distinct methodological assessment process. To develop EBs, NCCN expert panel members score a treatment regimen across five value domains: efficacy, safety, quality of evidence, consistency of evidence, and affordability, using a standardized 1 to 5 scale. The resulting value assessment output is a grid that reflects the average panel member score (rounded to the closest whole number) for each value domain.

Methodological Approach

The methodological approach comprises a value assessment tool's methodology, inputs, evidence standards, and transparency.

ICER's evidence reports and IVI's OSVP models both evaluate the comparative effectiveness and cost-effectiveness of health care interventions, but each organization employs a distinct methodological approach. ICER often assesses emerging products that are in the process of seeking U.S. Food and Drug Administration approval and, therefore, predominantly uses data from randomized clinical trials (RCTs) and network meta-analyses (NMA) to inform its comparative effectiveness analyses. ICER may also consider real-world evidence (RWE) as well as data submitted by manufacturers. IVI uses data from RCTs and RWE to inform its model inputs and also considers submitted data from manufacturers and other stakeholders.

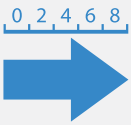
ICER and IVI assess interventions' incremental cost-effectiveness using cost-effectiveness models (CEMs). Access to ICER's model is limited; manufacturers with products under review can participate in ICER's model transparency program to gain temporary access to the model, which often requires a fee. In contrast, IVI publicly releases its model code and posts the R statistical package and detailed documentation on its website to allow for complete transparency and reproducibility.

Measures of efficacy or treatment effectiveness vary by assessment in both ICER evidence reports and IVI's OSVP models. ICER quantitatively incorporates measures associated with productivity and quality of life (QoL) in its CEM. Within the "Contextual Considerations and Potential Other Benefits" section of newer evidence reports, ICER includes qualitative discussions of select broader elements of value like acuity of need, magnitude of lifetime impact, patients' ability to achieve major life goals, caregiver and family QoL, complexity of regimen, and a treatment's ability to reduce health inequities. However, the extent to which these factors contribute to ICER's overall value assessment is minimal. Although the appraisal committee may vote on the degree to which these factors affect long-term value, these factors are not quantitatively captured in ICER's cost-effectiveness estimates. IVI's OSVP model inputs vary depending on the disease area under review; however, to date, IVI has quantitatively incorporated measures related to productivity and a treatment's effect on reducing a patient's earnings. IVI has also incorporated the value of hope, route of treatment administration, and measures related to variable patient response.

The methodological approach for NCCN's EBs is unique; EBs do not incorporate a formal comparative effectiveness analysis or a cost-effectiveness analysis. Instead, NCCN relies on expert panel members' clinical expertise and knowledge of the RCT evidence underlying NCCN's guidelines to make informed value judgments. Because the EBs reflect average scores across panel members and individual panel member scores are not known, it is not possible to reproduce the assessment.

Like IVI's OSVP models and ICER's evidence reports, NCCN's EBs consider the efficacy of a treatment, focusing mainly on the extent to which a treatment prolongs life, arrests disease progression, or reduces symptoms. EBs consider a treatment's safety profile, with treatments associated with fewer side effects scored higher, but they do not consider any additional patient-centered elements of value. EBs do not include a formal consideration of the costs associated with a treatment or regimen. Instead, panel members score the assessed intervention based on their knowledge of the overall cost of the regimen, including the cost of the drug, required supportive care, infusions, toxicity monitoring, management of toxicity, probability of care being delivered in the hospital, etc, with less expensive interventions earning a higher score.

Table 2. Examples of broader elements of value

Elements of Value	Description
 **Adherence-improving factors**	Advantages offered by an intervention that may improve patient adherence to treatment over existing alternatives (e.g., simpler dosing schedules, alternate routes of administration, or combination treatments)[21]
 **Equity**	Potential for a treatment to reduce important inequalities across racial, ethnic, gender, socioeconomic, or regional categories[22]
 **Insurance value**	Potential for a treatment to provide protection from physical risks of illness and financial risks of treating disease[22]
 **Productivity**	The effects of health improvement on productivity in the workplace or outside of it[21]
 **Real option value**	Potential for a treatment to extend life and create opportunities to benefit from other future advances in medicine[22]
 **Reduction of uncertainty**	New evidence that could better predict treatment outcomes[22]
 **Scientific spillover**	Potential impact a treatment could have on future research and development[22]
 **Severity of disease**	The severity (e.g., impact on length of life and/or quality of life) of a disease the intervention is intended to treat[22]
 **Value of hope**	Ill patients may be willing to accept greater risk to attain a chance at a better outcome (e.g., cure or extended survival), even if the plausibility of the better outcome is remote[21]

Assessment Process

This analysis considered the number of assessments conducted, as well as the processes for public comment, patient engagement, and updating assessments. Assessment counts were current as of December 2022.

ICER has completed 65 evidence reports and has four underway. ICER's evidence review process involves a scoping period, evidence synthesis, model development, draft evidence reports, a public meeting, and publication of a final report. ICER invites stakeholder engagement and public comment on the draft scoping document and draft evidence report and allows limited comment at the public meeting.

IVI has released two OSVP models and has one underway. IVI's process for developing OSVP models includes a conceptual framework, model scope, model protocol, model construction, and release of the open-source model. Stakeholders are afforded the opportunity to provide public comments on the model scope and model protocol, and after the release of each model.[12]

NCCN has embedded EBs in 61 NCCN guidelines. No public comment period is provided during NCCN's assessment process; however, stakeholders can submit data and request a change after an EB is published.

ICER and IVI both have processes for engaging patients in value assessment, but NCCN does not. ICER has a patient engagement program, which includes outreach to patients prior to topic selection and inviting select patient groups to participate in scoping conversations and the public meeting. To improve meaningful patient participation in ICER's processes, some patient groups have recommended that ICER modify its review timelines and processes to ensure patients, caregivers, and their representatives (who often have limited resources) have adequate time to provide input.[23]

IVI's approach to patient engagement follows an engagement checklist that adheres to its principles for partnering with patients.[24] Development of IVI's OSVP models involves conducting patient focus groups to inform model development, conducting qualitative research with patients to inform the model's value determinants and MCDA content, as well as administering a study quantifying the patient experience.

IVI's OSVP models are open to the public and allow end users to update the model with new data on a continuous basis. ICER updates its evidence reports occasionally as new evidence emerges and updates its value assessment framework every few years.[25] NCCN's guidelines are updated annually, but it is unclear how often individual EB scores within the guidelines are updated.

Identifying Key Challenges: Opportunities to Improve Value Assessment Methods and Tools

Since the emergence of U.S. value assessment tools less than a decade ago, the field has matured, and health care stakeholders are increasingly using assessment outputs to make insurance coverage and treatment decisions. However, current U.S. value assessment tools rely on a patchwork of diverse methodological approaches and processes, and assessing value within a complex, multipayer health system presents unique challenges that may limit the ability of these tools to conduct rigorous, reliable, and comprehensive value assessments.

Insufficient Consideration of Health Equity

It is important that assessments of value do not perpetuate or exacerbate disparities in health care access or quality. Previous research has identified that traditional value assessment methods like CEA may impose barriers to developing treatments for patients in disadvantaged communities as such value determinations are based on improving overall population health and assume all health gains are valued equally.[26,27]

In 2022, IVI and ICER announced efforts to refine their processes to support health equity goals in value assessment.[28,29] To successfully close current gaps, new methods for incorporating health equity in value assessments are needed. Additionally, the broader value assessment community must invest in research to identify and address drivers of health inequities and encourage representation of diverse populations in research to better inform health care decision-making.

Shortcomings in Existing Value Assessment Methods

Current value assessment tools employ differing methodological approaches, each with their own strengths and limitations. NCCN's EBs use a novel methodological approach to produce value assessments that reflect expert panel member opinions on treatment regimens. This approach limits the strength, transparency, and reproducibility of its assessments.

Alternatively, ICER evidence reports use more traditional value assessment methods including CER and CEA to produce population-level value assessments for health care interventions. However, previous research has identified limitations to traditional CEA approaches, including the inability to make context-specific value determinations and the difficulty associated with including non-standard elements of value.[30,31] These limitations may lead to value determinations that are not suitable for all decision contexts.[32]

Additionally, patients have unique clinical contexts, genetics, and preferences, which may prevent some patients from responding to a treatment that works well for the general patient population. Therefore, population-level assessments of health care interventions may undermine appropriate access to treatment for some patients. Given the associated implications for patient access, methodological transparency is critical. However, ICER's CEMs are not publicly available, undermining the ability for many assessment users to validate and replicate ICER's assumptions.

IVI's OSVP models combine more traditional CEA analyses with MCDA to allow end users to modify the weights of various value elements and the willingness-to-pay threshold. However, IVI has only published two models, which limits widespread stakeholder adoption and use.

To promote more comprehensive assessments of value, assessment tools should implement transparent methods that reflect real-world decision contexts to more reliably inform decision-making across stakeholders.

A Narrow Definition of Value Misaligned With Patient Needs

Each value assessment tool uses different inputs and parameters to produce a value determination for an assessed health care intervention. NCCN's EBs include an overall estimate of treatment efficacy, which reflects the treatment's ability to prolong life, arrest disease progression, or reduce symptoms as well as an estimate of the treatment's affordability. Despite being a shared decision-making tool, NCCN's EBs do not consider a treatment's effect on broader elements of value such as productivity or caregiver burden, two critical factors for patients with cancer.[32]

Value assessment tools, including ICER's evidence reports, that rely on QALY-based cost-effectiveness analyses may exclude broader value elements that matter most to patients,[21] resulting in health care decision-making guided by incomplete assessments of value that are misaligned with patient needs.[33] Moreover, health care thought leaders and governmental agencies, including the National Council on Disability, have identified ethical concerns related to the use of the QALY, in particular that QALYs place a lower value on treatments that extend the lives of people with disabilities and chronic illnesses.[17,34]

Although ICER evidence reports include a quantitative scenario assessment that incorporates a treatment's effect on productivity, other value elements are limited to a brief qualitative summary or are excluded from ICER's analysis altogether. In response, some patient groups have cited concerns with ICER's lack of inclusion of broader value elements in its CEMs and stated that critical elements of value have been omitted from ICER's assessments.[35] Previous research has also identified shortcomings in ICER's approach to patient engagement and incorporation of patient preferences into their analyses.[36-38]

Alternatively, IVI's OSVP models have quantitatively incorporated a wider range of value measures including those related to value of hope, route of treatment administration, and measures related to variable patient response, demonstrating that inclusion of broader value elements is possible.

Despite the progress made by current U.S. value assessment tools to capture broader elements of value, additional research is needed to develop methodologies that can incorporate these measures and advance tools that rely on a comprehensive definition of value aligned with patient needs.

Supporting Solutions: Investing in Transformational Research in the Field of Value Assessment

The PhRMA Foundation is taking a leading role in tackling challenges in the field of value assessment by investing in research on innovative methods that promote patient centricity and address longstanding issues related to health disparities and inequity.

The PhRMA Foundation launched its Value Assessment Initiative in 2017, as an extension of its over 20 years of health outcomes research funding.[39] Through this initiative, the Foundation funded the creation of four Centers of Excellence in Value Assessment and offered grant opportunities via Challenge Awards and Research Awards to support the work of pioneering researchers in this important and fast-growing field. This section highlights Foundation-supported research that has advanced progress in value assessment.

Striving to Improve Health Equity

The PhRMA Foundation supports vital new research into how value assessment methods and processes can better consider population diversity and drivers of health disparities.

Improving Access for Historically Underserved Populations

A core purpose of value assessment is to encourage the use of high-value treatments and services to promote more efficient health care delivery and improve system sustainability. The Research Consortium for Health Care Value, a Foundation-funded center of excellence, promotes the pursuit of value in health care delivery in the U.S. by identifying high- and low-value clinical services and helping to ensure that consumer preferences are incorporated in health care decisions. In a recent concept paper, researchers found that the burdens of low-value care may be especially high for historically underserved populations and that efforts to improve access to care and advance health equity will be most effective when done with the intention of promoting the provision of high-value care and minimizing low-value care.[40]

Incorporating Health Equity into Value Assessment

Numerous efforts are underway to improve how value assessment tools account for health disparities and inequity.[41] A recent study analyzed U.S. value assessment tools and identified examples where evidence on outcomes and preferences for value do not take diverse perspectives into consideration.[42] The authors proposed three approaches to explicitly incorporate health inequality impacts in value assessments of health care interventions: two-part health technology appraisal, distributional CEA, and equitable MCDA. To better incorporate health equity into existing value assessments, researchers recommend leveraging these methodological approaches within existing value assessment tools to capture measures related to health equity and incorporate input from patient populations on what endpoints matter most.

Advancing Rigorous and Transparent Value Assessment Methods and Tools

The PhRMA Foundation invests in research that tests and applies new and existing methodological frameworks to reliably incorporate patient-centered outcomes in value assessment for both population- and individual-level health care decision-making.

Generalized Risk-Adjusted Cost-Effectiveness (GRACE) Analysis

Value assessment thought leaders Charles Phelps, PhD, and Darius Lakdawalla, PhD, developed GRACE analysis, a novel approach to CEA that aligns economic assessments of treatments with patient preferences and experience of care.[43] In GRACE analysis, differential cost-effectiveness thresholds (relative to traditional CEA) are applied based on disease severity (e.g., higher thresholds for more severe diseases) and other patient circumstances to better recognize the value of treatments that promote equity and significantly improve patient QoL.

Multi-Criteria Decision Analysis

MCDA is a decision-making method that systematically weighs various value elements that may fall outside traditional value assessments, such as a treatment's scientific novelty, a patient's disease severity, or how a treatment may affect a caregiver's productivity.[44] When applied to value assessment, this customizable methodological approach allows for more nuanced examination of how perceptions of value may differ across stakeholder groups, including patients, payers, and providers.

A growing body of empirical evidence highlights successful applications for MCDA in value assessment. In a recent study, researchers at the Center for Pharmaceutical Value (pValue), a Foundation-funded center of excellence at the University of Colorado's Anschutz Medical Campus, examined patient and payer preferences on the importance of additional value elements (e.g., real value option and value of hope) and assessed the potential for these elements to be incorporated in MCDA.[22] The study highlighted how the implementation of MCDA can capture additional value elements that matter to patients but have traditionally been difficult to measure and excluded from systematic assessments of value.

Additionally, a recent systematic literature review of MCDA investigated the criteria and scoring functions applied in value assessment tools for rare disease therapies to gain a better understanding of commonly referenced novel value elements.[45] Researchers found that MCDA is a promising tool for capturing novel value elements related to orphan drugs and could be used to supplement traditional CEA as measurement methods and scoring functions for novel value elements mature.

Value Assessment Methods for Genetic Testing and Diagnostics

Current U.S. assessment tools predominantly examine the value of biopharmaceutical health care interventions. Historically, it has been difficult to assess the relative value of other health care interventions, including surgery and diagnostics, due to significant gaps in reliable clinical evidence. To address this challenge, researchers are identifying new applications for value assessment of non-pharmaceutical health care interventions. For example, researchers identified a new methodology for developing novel clinical and patient-reported measures to capture the informational value of genetic tests.[46] Application of the tool would allow end users to conduct a comparative effectiveness assessment to examine the relative value of genetic tests. Additionally, a recent study examined the current methodological challenges associated with incorporating genetic testing in economic evaluations, including those related to study design, costs, measurement and valuation of health outcomes, and modeling.[47] The researchers found that new methods are needed to advance the inclusion of genetic testing in economic evaluation.

Broadening the Definition of Value and Prioritizing Patient Focus and Engagement

The PhRMA Foundation also funds research that strives to close gaps in the collection and use of patient-centered outcomes and to advance value assessment methods that prioritize patient focus and meaningful patient engagement.

Advancing Patient-Informed Value Elements

Few elements of value are derived solely from the patient perspective. To overcome this challenge, researchers at Patient-Driven Values in Healthcare Evaluation (PAVE), a Foundation-funded center of excellence at the University of Maryland, are engaging directly with patients to understand their values, experiences, and treatment preferences to advance value assessments that reflect the individualized health care needs of patients. For example, a recent PAVE study systematically engaged patients to identify a set of patient-informed value elements and demonstrated the potential for such elements to be incorporated into existing value assessment tools and economic evaluations to improve data-generation and decision-making processes.[48]

PAVE researchers are also working directly with patients to elicit feedback and gain insights on patient experience and how to measure patient outcomes, which can feed into how treatment effectiveness is evaluated. A 2019 analysis by PAVE employed a patient-centered approach to assess the cost-effectiveness of hepatitis C treatments and found that the assessed treatment was cost-effective from a health sector perspective and cost-saving when including non-health costs such as patient/caregiver time and productivity.[49]

Identifying New Methods to Incorporate Patient-Centered Outcomes in Value Assessment

Researchers at the Center for Enhanced Value Assessment (CEVA), a Foundation-funded center of excellence housed in Tufts' Center for the Evaluation of Value and Risk in Health, are exploring enhanced cost-effectiveness elements and engaging patients and stakeholders to identify important novel and non-standard value elements. A recent analysis summarized the evolving landscape of this research, highlighting noteworthy progress in identifying rigorous theoretical and mathematical foundations for certain novel value elements including insurance value, real option value, value of hope, and value of knowing.[50] Some international HTA bodies, including the National Institute for Health and Care Excellence in the United Kingdom and Sweden's Dental and Pharmaceutical Benefits Agency, are already considering certain additional value elements (e.g., severity modifiers to cost-effectiveness thresholds), but other elements have yet to gain traction and additional research is needed to support greater inclusion of value elements important to patients.

Researchers are also testing how broader value elements can be incorporated in disease-specific value assessments. A recent CEVA study examined how the inclusion of broader elements of value like productivity and caregiver burden can influence cost-effectiveness estimates.[51] The authors conducted two case studies focused on human papillomavirus (HPV) infection and adult early-stage Hodgkin's lymphoma and found that incorporating broader elements of value can substantially influence cost-effectiveness, although their magnitude and direction can differ across intervention and disease context. The results highlight the potential impact of harnessing broader value elements and the need to generate more empirical data on these value elements.

Quantifying the Value of Hope

"Value of hope" is commonly cited as an important patient-centered value measure and refers to the idea that severely ill patients may be willing to accept greater risk to attain a chance at a better outcome (e.g., cure or extended survival), even if the plausibility of the better outcome is remote.[21] Researchers are exploring how empirical evidence on the value of hope can be incorporated into value assessment.

A recent study aimed to generate quantitative data on the value of hope by estimating money-equivalent values for cancer treatments offering hope, adjusting for variation in patients' financial resources and preferences.[52] The study results demonstrate that it is possible to generate empirical evidence to support the value of hope as an additional value element, but variation in patient valuations and preferences may limit the applicability of this measure in population-based value assessments.

Developing Patient-Centered Real-World Evidence

Current value assessment tools predominantly rely on evidence from RCTs to inform evaluations of treatment efficacy, but such assessments may omit important context surrounding patients' experience of care in the real world. Researchers are pursuing opportunities to generate value assessments that more accurately reflect patients' preferences and lived experience through the incorporation of RWE.

A recent study convened a multidisciplinary advisory board to develop consensus on recommendations for how patient experiences/insights data can be incorporated into the design, conduct, and translation of real-world research into applications such as value assessment.[53] Future application of the recommendations identified in this study can enhance the patient focus of real-world data collection and use, as well as health care research and delivery more broadly.

Looking Ahead

With support from the PhRMA Foundation, health care researchers are advancing the field of value assessment through the development of new methods and approaches that incorporate patient-focused value elements and equity in health care decision-making. To support the use of value assessment tools that are rooted in rigorous, reliable, and patient-centered methods, this work must continue to integrate scientific discovery and social sciences to promote appropriate valuations of health care interventions.

Further advancements in the field of value assessment will require multistakeholder collaboration and sustainable funding streams. To meet this challenge, the PhRMA Foundation will continue to invest in new and innovative research and provide opportunities for researchers at every stage of their careers to focus attention on these research needs.

The Foundation's re-envisioned Value Assessment and Health Outcomes Research Program provides awards for predoctoral students, postdoctoral trainees, and early- and mid-career faculty. The Foundation will also launch a new Frontier Award designed to encourage additional empirical research that takes a more holistic view of how treatments should be valued.

These funding streams, when complemented by additional dedicated funding from other groups like the National Institutes of Health (NIH), the Agency for Healthcare Research and Quality (AHRQ), the Patient-Centered Outcomes Research Institute (PCORI), and other foundations, will foster opportunities to initiate or expand efforts to address persistent gaps in value assessment.

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Appendix

The PhRMA Foundation supports innovative research that addresses challenges in assessing the value of health care interventions. Through its Value Assessment Initiative, the Foundation funded the creation of four Centers of Excellence in Value Assessment and offered grant opportunities via Challenge Awards and Research Awards to support the work of pioneering researchers in this important and fast-growing field. Please see below for a list of research publications from the Centers of Excellence, Challenge Award recipients, and Research Award recipients. Publications with an * indicate collaboration across centers. To avoid redundancy, publications are categorized by the lead author's affiliation.

CENTERS OF EXCELLENCE IN VALUE ASSESSMENT

Center for Enhanced Value Assessment (CEVA)

CEVA, headquartered within the Center for the Evaluation of Value and Risk in Health (CEVR) at Tufts Medical Center, aims to explore the incorporation of non-traditional elements of value into cost-effective analyses. CEVA's efforts involve engaging stakeholders — including patients, health insurers, and therapeutic area leaders — to identify important novel and non-standard elements to inform coverage, reimbursement, and access decisions.

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Center for Pharmaceutical Value (pValue)

pValue, headquartered at the University of Colorado School of Medicine, applies and tests novel methods, such as multi-criteria decision analysis (MCDA), to improve the pharmaceutical coverage and reimbursement decision-making process. pValue pilot experiments help determine where expanded decision tools should be used alongside existing approaches to maintain transparency and consistency while introducing more fairness in pharmaceutical coverage and reimbursement decision-making in the U.S.

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Patient-Driven Values in Healthcare Evaluation (PAVE)

PAVE is a unique collaboration between the University of Maryland School of Pharmacy, the National Health Council, patient community leaders, and payer and industry leaders. PAVE is dedicated to developing and advancing new methods to incorporate the patient perspective into value assessment and value-based decision-making. PAVE is building a diverse and extensive network of partners to build technical expertise in patient-centered health outcomes research, education, and dissemination.

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CHALLENGE AWARDS

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