Policy Brief

Alternatives to QALY-Based Cost-Effectiveness Analysis for Determining the Value of Prescription Drugs and Other Health Interventions

National Council on Disability
November 28, 2022
Policy Brief: Alternatives to QALY-Based Cost-Effectiveness Analysis for Determining the Value of Prescription Drugs and Other Health Interventions

National Council on Disability, November 28, 2022
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November 28, 2022

The President
The White House
Washington, DC 20500

Dear Mr. President:

On behalf of the National Council on Disability (NCD), I am pleased to submit this policy brief, titled *Policy Brief: Alternatives to QALY-Based Cost-Effectiveness Analysis for Determining the Value of Prescription Drugs and Other Health Interventions*.

This brief supplements our 2019 report, *Quality Adjusted Life Years and the Devaluation of Life with a Disability*, which described the design and discriminatory impact of QALY-based cost-effectiveness analysis (Standard CEA) – a method of comparing the cost and effectiveness of two or more healthcare interventions and determining if the value of an intervention justifies its cost. Its goal is to inform decision-makers about the allocation of healthcare resources. The report described how use of the QALY in Standard CEA results in undervaluing prescription drugs that extend the lives of people with disabilities, resulting in restricted insurance coverage in countries where it is commonly utilized to inform coverage decisions. The report further described how the discriminatory impact of the QALY on patients overseas led to its prohibition in the United States and to the Affordable Care Act of 2010 prohibiting the Secretary of the Department of Health and Human Services from using the QALY, or similar measure, to determine coverage, reimbursement, or incentive programs under the Medicare program. Currently, however, state Medicaid programs have no prohibition on referencing QALY-based value assessments of new drugs and treatments, and pharmacy benefit managers, companies that manage prescription drug benefits on behalf of health insurers, reference these assessments when negotiating the prices they will pay for prescription drugs.

Health economists have recognized the ethical concerns and discriminatory aspects of the QALY in Standard CEA regarding people with disabilities, and several novel methodologies have been developed that aim to address these deficiencies. This policy brief describes these alternatives, their strengths and weaknesses; highlights organizations that are using or implementing alternative methodologies for valuing health interventions; and makes recommendations for further research and further development of alternative methodologies. The methodologies described in this brief are: Equal Value of Life Years Gained (eqVLYG); The Efficiency Frontier (EF); Health Years in Total (HYT); Burden Augmented by Deadliness and Impact (BADI); Multi-Criteria Decision Analysis (MCDA); and Generalized Risk-Adjusted Cost-Effectiveness (GRACE) framework. They utilize a modified QALY, a QALY alternative, or avoid the QALY altogether. Each is an example of movement toward a new era in value assessment. Also described is Augmented or Extended Cost-Effectiveness Analysis, which is Standard CEA with added considerations of what patients value.
The efficient use of healthcare resources is an important goal for the United States. When assessing value of drugs and treatments there are many considerations that impact value. Healthcare equity should be at the forefront of decisions on which methodologies to employ, and continued investment should be made to evaluate and strengthen the methodologies that seek to achieve it.

NCD appreciates your commitment to addressing healthcare disparities and ensuring equity in healthcare. We hope that the information in this brief can help further support those efforts.

Respectfully,

Andrés J. Gallegos J.D.
Chairman

(The same letter of transmittal was sent to the President Pro Tempore of the U.S. Senate and the Speaker of the U.S. House of Representatives.)
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The purpose of this brief is to present an overview of methodologies that seek to correct the distributional issues raised by standard [cost-effectiveness analysis (CEA)] by promoting equity and nondiscrimination. It highlights their strengths and weaknesses and makes recommendations aimed at federal investment in continued research to further develop these methodologies.
Background

In NCD’s 2019 report on quality-adjusted life-years (QALY), we critiqued the use of standard cost-effectiveness analysis (CEA) to determine the value of prescription drugs and treatments because of its use of the QALY. As described fully in that report, standard CEA utilizes the QALY in an analysis that compares the benefits and values of different drugs and treatments, and the results are used to inform the allocation of health care resources. Our critique focused on the fact that this analysis puts lesser value on drugs and treatments that extend the lives of people with disabilities as compared to the lives of people without disabilities or chronic illnesses. We highlighted the fact that this methodology has resulted in restricted access to drugs and treatments for people with disabilities and chronic illnesses in countries where it is applied in nationalized health care systems, like NICE in the United Kingdom. Additionally, the data underlying the QALY, as utilized in standard CEA, is not reflective of patients’ experiences with illness and disability, contributing to standard CEA’s devaluation of life-extending drugs and treatments.

We recommended that both federal and private health insurers utilize, or reference, value assessments determined through alternative methods—ranging from well-established methods already used by federal agencies, such as cost-benefit analyses, to promising alternatives that use patient preferences to determine the value of prescription drugs and health care treatments and could be used in a nondiscriminatory manner. We recognized that alternatives may themselves be discriminatory if used in certain contexts, or if they are used without paying sufficient attention to the possibility that discrimination may occur.

Several alternatives to standard (QALY-based) CEA have been developed in response to criticism that standard CEA is incomplete, for example, it is focused on the health sector perspective, undervalues life extension for people with disabilities, and does not consider the societal perspective or patient perspectives. The purpose of this brief is to present an overview of methodologies that seek to correct the distributional issues raised by standard CEA by promoting equity and nondiscrimination. It highlights their strengths and weaknesses and makes recommendations aimed at federal investment in continued research to further develop these methodologies.

A. Alternatives to Standard Cost-Effectiveness Analyses (CEA)

Alternative methodologies to standard CEA differ from one another in a variety of ways, including whether they serve all of the same functions as...
standard CEA, whether the alternative has been used in practice or is theoretical, and what data underpins the method. Some of the alternatives rely on the QALY but apply it differently than does standard CEA. The order of alternatives presented does not indicate importance or level of efficacy.

**i. Equal Value of Life Years Gained (evLYG)**

In response to concerns that the QALY does not give the same weight to gains in the length of the life of people with chronic illnesses and long-term disabilities provided by a drug or treatment, as it does healthier populations, in 2018, the Institute for Clinical and Economic Review (ICER) introduced the equal value of life years gained (evLYG) metric as an alternative to the QALY, and has since incorporated it in its 2020–2023 Value Assessment Framework to be a component of all their new health technology assessments (HTAs). According to ICER, the evLYG, “evenly measures any gains in length of life, regardless of the treatment’s ability to improve patients’ quality of life. In other words, if a treatment adds a year of life to a vulnerable patient population—whether treating individuals with cancer, multiple sclerosis, diabetes, epilepsy, or a severe lifelong disability—that treatment will receive the same evLYG as a different treatment that adds a year of life for healthier members of the community.”

The evLYG is a modification of the Equal Value of Life (EVL) approach, which was introduced in 1999 to address societal concerns with the QALY. Under EVL, any extension of life is valued at a quality-of-life weight of 1 (the highest value possible); however, it has been criticized for undervaluing interventions that both extend life and improve quality of life (QOL). Under ICER’s modified version of the EVL approach, the evLYG, any extension of life is valued the same across every population, at a quality-of-life weight of the general population.

A 2021 report by a disability advocacy organization critiqued evLYG as not going far enough; opining that while evLYG eliminates the risk of undervaluing life-extension for people with disabilities, it affords no value to quality-of-life improvements. In contrast, a 2021 examination of how the evLYG works, conducted by health economists, found that evLYG does capture those improvements as well, explaining, “Arguably, the name ICER chose for this outcome is a misnomer, as it implies that there is no quality-adjusted component; however, that would be an incorrect assumption, as evLYs do include improvements in quality of life.” ICER clarifies that the evLYG assigns the same quality-of-life weight during any extension of life while also allowing for quality-of-life improvements during all other time periods (i.e., time observed while on standard of care).

**ii. Health Years in Total (HYT)**

In 2019, researchers from the University of Washington introduced the Health Years in Total (HYT) method, which seeks to address the shortcomings of the QALY, the EVL, and the evLYG. HYT utilizes a modified QALY and the researchers assert that HYT enables patients with “lower quality of life” to fully benefit from interventions that extend life expectancy and that HYT may provide a viable alternative to QALY and evLYG. They advocated for further “critique, development, application and testing of the HYT framework.” HYT separates life expectancy effects from QOL impacts using an additive, rather than multiplicative, approach (Figure 1).
HYT has limitations. It does not directly address distributional issues regarding whether populations with higher QALY shortfalls or other equity factors should receive more resources. However, explicit independent methods to address the distributional problems can be applied to these metrics. More research is needed to understand HYT’s impact on technology prioritization and the appropriate value or budget thresholds. One critique called the HYT framework a “poor substitution” for the QALY, acknowledging the metric reduces distributional inequity “but does not altogether fix it.”

Disability rights advocates have described HYT as a better approach to standard CEA because it removes the devaluation of life extension of people with disabilities. Though there are positive attributes to HYT, one shortcoming among all the utility-based metrics is that they have often relied on utility values generated based on the general population’s preferences, although it can incorporate utilities derived from either patients or general population preferences. A ‘utility value’ is a number between 0 (death) and 1 (perfect health) that individuals assign to a health state.

Figure 1. Health years in total (HYT) framework and contrast with the traditional quality-adjusted life-year (QALY) and the equal value of life (EVL) frameworks.
iii. The Efficiency Frontier (EF)

The Efficiency Frontier (EF) is an alternative to cost-effectiveness analysis that makes use of condition-specific measures. It benchmarks the price and benefit afforded by a new drug to the value provided by existing drugs. The particular outcomes and costs of existing drugs are displayed in an efficiency plot—costs on the horizontal axis and value of benefits on the vertical axis. The efficiency of the new drug in a “cost per unit of benefit” is then compared to that of the most efficient existing treatments (i.e., those on the frontier). This helps to establish a maximum reimbursement rate for a particular treatment by deriving the price at which it would be just as efficient. Treatments that are demonstrated to be below the frontier require further justification for reimbursement at that price, or a price reduction. Of note, the entire process is carried out for a specific indication—there is no attempt to somehow prioritize across indications (Figure 2).

In our 2019 report, we highlighted that the German Institute for Quality and Efficiency in Health Care had adopted the efficiency frontier. The main benefit of the efficiency frontier approach is that it is clear, easy to use, and transparent. Additionally, it does not require the use of QALYs as the measure of a treatment’s benefit. By using existing, condition-specific

![Efficiency Frontier Diagram](image)

Figure 2. The horizontal gradient (=0) indicates no efficiency while the vertical gradient (=90) indicates infinite efficiency. Positive gradients (e.g., between points 6 and 7) reflect additional benefit for increased cost. Negative gradients (e.g., between points 6 and 7) indicate less benefit at more cost.
clinical metrics as its measure of value, the efficiency frontier does not need the QALY’s EQ-5D-derived utilities and thus avoids their discriminatory assumptions. For the same reason, it does not require the development of alternative, disability-friendly health utilities.\textsuperscript{18}

Though this is an improvement over traditional cost-effectiveness analysis that uses the QALY, the efficiency frontier has its own drawbacks. It looks at either one benefit of a health care treatment at a time (implying multiple frontiers), or aggregation of different benefits into a single score.\textsuperscript{19} Moreover, because it is based solely on existing clinical metrics, it cannot account for benefits provided by a new treatment that are not incorporated into such an existing measure. The efficiency frontier does not allow for direct comparisons across conditions, and its reliance on the efficiency of existing drugs means it cannot be used directly to assess the value of the first drug in a therapeutic area. The frontier may be less applicable if there has not been recent pharmaceutical innovation in that therapeutic area.\textsuperscript{20} Further empirical testing of its feasibility and usefulness is required to build on existing examples where it has been applied.\textsuperscript{21} Additionally, as with any other cost-effectiveness metric, its relevance depends on having the condition-specific information needed to measure outcomes that matter to people living with the condition being treated.

\textit{iv. Generalized Risk-Adjusted Cost-Effectiveness (GRACE)}

The Generalized Risk-Adjusted Cost-Effectiveness (GRACE) framework is a generalization of traditional cost-effectiveness analyses that eliminates the need to assume constant returns to health-related quality of life under traditional (QALY-based) CEA methods.\textsuperscript{22} It was developed to respond to the QALY’s discriminatory aspects.

The authors of the GRACE framework demonstrate mathematically that the method implies cost-effectiveness thresholds become more generous for more severe illness.\textsuperscript{23} Therefore, this method shows more worth to quality-of-life gains for people with disabilities.\textsuperscript{24} The framework seeks to incorporate uncertainty in health outcomes into the model using a “Generalized risk-adjusted QALY.” The formula used to compute cost-effectiveness in the GRACE framework implies people with “lower quality of life” are more willing to trade life expectancy for improved quality of life—it therefore accommodates permanent disability in its formulation as well as a wider range of risk preferences. The model has evolved over time, with a recent article noting that “under GRACE, disability always increases the [willingness to pay] for [quality-of-life] gains.”\textsuperscript{25} By incorporating the patient-preference perspective that quality-of-life gains are more highly valued than life-year extensions for certain people with disabilities, this new framework will encourage more investment in rigorous patient preference studies and in technologies addressing unmet needs for people with disabilities.\textsuperscript{26}

The GRACE framework comes with its own shortcomings, underscoring the continued need for better data, including new estimates of attitudes toward risk in health outcomes. Like traditional CEA, the GRACE method currently relies on health utilities of a “representative individual” that do not necessarily incorporate issues such health inequities, on which topic the authors suggest future research. In a forthcoming book from Oxford University Press,\textsuperscript{27} the GRACE framework has been extended to show that
treating people with disabilities has increasing value as the extent of a disability increases. Further analysis of the GRACE framework is needed to understand what discriminatory attributes of QALYs may continue to be considered in the framework’s formula. We caution that it is important to consider that patient preference data on how people value quality-of-life gains versus life-year extensions must be applied in a manner that accurately reflects the differences in values among people with differing disabilities and coexisting conditions. As with the HYT framework, the GRACE framework would benefit by use of health utilities that are based on more fulsome survey instruments and with the input of a broader group of stakeholders, including people with long-term disabilities and those with chronic illnesses.

v. Burden Augmented by Deadliness and Impact (BADI)

The Burden Augmented by Deadliness and Impact (BADI) measure of health benefit is an alternative to the QALY that seeks to address how a disease reduces life span (deadliness) and increases the negative consequences on quality of life (impact). Both are quantified in relation to the burden of senescence, also known as aging. As described in the literature, the measure of “deadliness” gives priority to urgent, potentially fatal conditions and to diseases manifesting earlier in life, while the measure of “impact” captures reduced quality of life beyond that which is expected with aging. BADI does not use the QALY. It takes the same two dimensions in the QALY and decompartmentalizes them, looking at relative (not absolute) improvements
from the current standard of care. In an interview with Dr. Jaime Caro, developer of the BADI and the efficiency frontier, he described the BADI as being “completely non-discriminatory because it only deals with the impact of that disease,” and the method “removes a lot of the problems with the QALY.”

The BADI is described as an improvement in value frameworks centered on what is important to patients. It is credited for addressing disease impact, accounting for impacts that are deadly and not deadly, not imposing a trade-off between prioritizing the likelihood of a condition to be fatal versus the quality-of-life impact of the disease and applying to any disease or intervention.

However, while the BADI approach is novel and merits further exploration, it does not yet offer a specific tested method. It is yet to be established for most diseases, nor have practical ways to measure the effectiveness of interventions (especially on impact universally) been developed. More research on the impact of aging without disease would be very helpful and experience with this measure must be accumulated to describe its practical limitations and any distributional implications.

B. Augmented or Extended Traditional CEA

Standard CEA is conducted from a health care sector perspective, a viewpoint for conducting a cost-effectiveness analysis that includes formal health care sector (medical) costs borne by third-party payers and paid out of pocket by patients. But cost and value may also come from other sources and accrue to multiple stakeholders. Some health economists have advanced the idea of augmenting or extending standard CEA (ACEA/ECEA) to include nonmonetary elements of value to patients, rather than replacing the QALY.

In 1996, the U.S. Public Health Service’s Panel on Cost-Effectiveness in Health and Medicine established a set of standard methods to improve the quality and comparability of CEAs. They recommended using the QALY (health sector perspective) as a health outcome measure in CEAs and also as a societal perspective—a viewpoint for conducting a cost-effectiveness analysis that incorporates all costs and health effects regardless of who incurs the costs and who obtains the effects. The societal costs proposed were the cost of health care services; costs of patient time expended for the intervention; costs associated with caregiving; other costs associated with illness, such as child care and travel expenses; economic costs borne by employers, other employees, and the rest of society, including so-called friction costs associated with absenteeism and employee turnover; and costs associated with non-health impacts of the intervention, such as on the educational system, the criminal justice system, or the environment.

Twenty years later, the Second Panel on Cost-Effectiveness in Health and Medicine was convened to provide an updated guideline reflecting methodological advances and consideration of ethical and distributional issues. It found that since publication of its recommendations in 1996, there had been a substantial increase in the number of published CEAs, and many had not used a societal perspective as defined by the original panel. One study found, for example, that only 341 (29 percent) of 1,163 QALY analyses published through 2005 adopted a societal perspective.
recommended, once more, that CEAs reference the health care sector perspective and a societal perspective.\textsuperscript{37}

In addition, between 2011 and 2018, there were several studies suggesting that the weakness of standard CEA would be improved by incorporating societal values, such as the value of knowing and the value of information.\textsuperscript{38,39}

For example, patients value treatments that provide “option value,” the value to patients that some treatments allow them to survive until the discovery of even more effective treatments.\textsuperscript{40,41} Patients also value “hope,” the added value placed on treatments that increase not just median survival, but also “tail of the curve” survival.\textsuperscript{42,43}

In 2018, a Special Task Force of the Professional Society for Health Economics and Outcomes Research (ISPOR) published a report with the purpose broadening the view of what constitutes value in health care and to spur new research on incorporating additional elements of value into CEA.\textsuperscript{44} Twelve potential elements of value were considered. Four of them, QALYs, net costs, productivity, and adherence-improving factors, are conventionally considered in value assessments. Eight others, which would be more novel in economic assessments, were discussed: reduction in uncertainty, fear of contagion, insurance value, severity of disease, value of hope, real option value, equity, and scientific spillovers.\textsuperscript{45}

The authors noted that most of these are theoretically well understood and available for inclusion in value assessments, but equity and scientific spillover effects are not and will require more theoretical development and consensus. The task force believes that considering these additional elements would result in a more comprehensive CEA and that possible approaches include integrating them as part of a net monetary benefit calculation, including elements as attributes in health state descriptions, or using them as criteria in a multicriteria decision analysis.\textsuperscript{46}

A 2018 study put this enhanced version of CEA into practice by creating a cost-effectiveness analysis that included factors of value to patients and society.\textsuperscript{47} In addition to the traditional payer components (medical costs; adverse events; survival benefit; quality of life/patient-reported outcomes), researchers included societal components, including productivity loss, nonmedical costs (transportation, patient time costs), caregiver burden, option value of treatment, and the value of hope. The study indicated more net monetary benefit when incorporating broader societal components, but due to limitations in the research, more studies are needed to fully develop this concept. It will be important in building and exploring this type of methodology to advance parallel efforts to develop better health state utilities and patient-reported outcomes data to use within the models.

C. Multi-Criteria Decision Analysis

Multi-Criteria Decision Analysis (MCDA) is neither augmented nor enhanced CEA, nor a method that does not utilize the QALY. Rather, it is a methodology that acknowledges the complexity of health care decision making and responds to concerns that CEA is incomplete—particularly for factors such as equity and fairness in determining value. MCDA allows varied decision makers to consider many distinct factors relevant to a health care decision—such as cost, clinical outcomes, and
administrative burdens—and determine how important each of these factors is to them. Its focus is eliciting decision makers’ preferences in order to create decision weights. It then measures performance of each intervention candidate along each specified criterion that formally shows the trade-offs among them. For example, a payer using MCDA would first rank each factor that is relevant to the decision against one another. Each criterion would then be given a weighted “score” representing that criterion’s importance to the decision maker ranked in the order of importance, sometimes with as many as 15 criteria. Next, researchers would compare how each of the treatments being considered relate to one another, such as for clinical outcomes and costs. Depending on the decision to be made and the criteria being assessed, the method generates a single average weighted score for each treatment that is the aggregate of both how the treatment scores on each of the criteria and how important those criteria are to the decision maker, which then shows the relative value of the treatments to one another.

A particularly strong asset of MCDA is transparency to the public regarding the data used in assessing value and the decision making process. MCDA prioritizes gathering data on what is important to improving the decision, driving investments in data improvement. It can be used at various levels of decision making, from choices on what should be covered benefits, to helping people choose among therapies, to informing choices among competing health plans.
A potential problem with MCDA is the use of standard CEA-based methods to measure QALY gains for disabled people as one of the multicriteria inputs. Experts on MCDA and health economics organizations agree that the MCDA can and should use QALYs as measures of health. Because MCDA allows societally determined weights to emphasize the differential value of health gains for various subgroups, when properly used, it can approach the goal of patient-centered measures of value, even with use of the QALY. To do this, the MCDA model would measure QOL and life extension (LE) gains for different groups of people and would include higher weights on health gains for people related to the extent of disability. An ISPOR task force has also stated that specific measures of health gains such as QALYs are necessary core elements in any broader MCDA model of value in health care but emphasizes that health gains to different subpopulations can be included in MCDA measures with higher weight given to subgroups of interest, unlike QALY application in standard CEA.

The Innovation and Value Initiative (IVI) specifically includes QALYs in its use of MCDA. The IVI, an organization that focuses on patient-centered outcomes and preferences in value assessment models and that advocates for transparent, open-source model development, includes the CEA-based measure of QALYs as an outcome in its open source value models, while recognizing limitations and concerns about discrimination against people with disabilities. IVI explains that despite concerns about this use of the QALY measure, “including QALY as a metric in the model, along with many other key outcomes, will allow the flexibility to understand and evaluate the importance of looking at a wide range of outcomes and will allow comparison with prior economic evaluations that have used this metric.”

D. Frameworks for Determining Value to Individual Patients
i. Shared Decision Making: Costs and Trade-Offs of Health Care at the Individual Level

Unlike the other methodologies discussed, which all work at a population level to determine clinical and cost-effectiveness, shared decision making is a process that is between doctors and their patients: an exchange of information, values, and preferences between beneficiary and practitioner to arrive at a treatment decision that is based on the beneficiary’s values and preferences. It is a strategy for improving an individual patient’s health care decisions that also has potential for cost savings by improving the patient’s health outcomes, reducing expensive adverse events from failed treatment, and eliminating care that people do not want or need. The Affordable Care Act (ACA) required U.S. Health and Human Services (HHS) to establish a shared decision making program with the purpose of collaborative processes between patients, caregivers or authorized representatives, and clinicians that engages them in decision making, provides them with information about trade-offs among treatment options, and facilitates the incorporation of patient preferences and values into the medical plan. It requires HHS to contract with an entity to establish consensus-based standards for patient decision aids for preference sensitive care and a certification process for patient decision aids for use in the
federal health programs and by other interested parties. In the past 10 years, the HHS Center for Medicare and Medicaid Innovation (CMMI) has financially supported the shared decision making program. It is unclear whether it has established a certification process for patient aids and if patient decision aids are being used in federal health programs, for example, Medicare and Medicaid.

Shared decision making using patient decision aids is a practice in health care organizations across the United States and both Medicare and Medicaid beneficiaries are likely participants. In 2018, the National Quality Forum published the National Quality Partners Playbook™: Shared Decision Making in Healthcare (hereafter Playbook) defining standards for health care stakeholders to reference how to achieve high-quality shared decision making using high-quality patient decision aids such as those being developed by the Innovation Center. The Playbook gives examples of how health care organizations are integrating and improving shared decision making in clinical practice. In one example, “men diagnosed with an enlarged prostate used online decision aids to learn more about which treatment options are most suited to their care preferences before meeting with a urologist. After analyzing data from patients, the urology department changed its process to offer patients who expressed a preference for nonsurgical care the choice either to see a urologist for specialized care or return to their primary care physician for follow-up care.”

As of February 2022, the Patient-Centered Outcomes Research Institute (PCORI) has funded 55 comparative clinical effectiveness research studies related to shared decision making and seven implementation projects that seek to integrate effective shared decision-making approaches in health care settings and help patients and their clinicians make choices that are best for them.

**ii. Patient Preference Information: Stated Preference**

Patient preference information is an essential component of both value assessment and shared decision making in health care if it is to reflect value to patients. ISPOR acknowledges that “accurate and meaningful measures of patient-centered outcomes and preferences are critical to numerous decisions throughout product development, clinical research, regulation, technology assessment, and healthcare delivery,” and there is a need for research to improve the methods for translating outcomes related to a disease or condition into utilities, especially for people with disabilities.” ISPOR recognizes that stated-preference research is useful for value judgments about different treatments and their health outcomes. In fact, ISPOR’s recently updated Consolidated Health Economic Evaluation Reporting Standards (CHEERS) statement—created to ensure health economic evaluations are able to be identified, interpreted, and useful for decision making—states that patient perspectives are needed from the outset of value assessment. PCORI and other entities have funded research related to patient preferences. Methods to collect patient preference information indicate that it can be done in a manner that is systematic, with consistency of preferences for certain treatment attributes, though more research is needed across patient groups. Patient preferences can be integrated into
clinical effectiveness studies to improve their responsiveness to patient values.\textsuperscript{71}

\textbf{E. Organizations Conducting Value Assessments or Advancing Methodologies Outside QALY-Based CEA}

\textit{i. Patient-Centered Outcomes Research Institute (PCORI)}

PCORI was authorized to be established in 2010 as part of ACA to fund comparative clinical effectiveness research with a duty to identify national priorities that consider patient needs, outcomes, and preferences in its work.\textsuperscript{72} Since its establishment, PCORI has become the leader in developing strategies for patient engagement in research to assure that its funded studies capture outcomes that matter to patients. In 2019, PCORI’s funding was reauthorized by Congress. As part of the reauthorization, Congress mandated that PCORI-funded research be designed, as appropriate, to capture “the full range of clinical and patient-centered outcomes,” including “the potential burdens and economic impacts” of the utilization of medical treatments, items, and services.\textsuperscript{73}

PCORI is prohibited from developing or employing the QALY (or similar measure that discounts the value of life because of an individual’s disability) as a threshold to establish what type of health care is cost effective or recommended and does not fund studies that conduct comparative cost-effectiveness analysis. PCORI will fund studies that directly compare patient and/or family cost burden as secondary outcomes, providing specific examples such as out-of-pocket costs, time costs associated with an approach, and financial toxicity. PCORI-funded studies capture perspectives of people living with disabilities and chronic conditions, which could be used to improve the accuracy of health utilities used in value assessments—a critical need for non-QALY methods. Burden and economic impact data collected and analyzed as part of PCORI-funded studies can be useful to improve decisions made by patients and their providers. It could also add quality data to help define how a treatment impacts a patient’s QOL. This data is also useful for the development of shared decision making tools and generation of patient-reported outcome measures.

\textit{ii. Innovation and Value Initiative (IVI)}

IVI is a nonprofit research organization that invests in research that emphasizes patient perspectives on value and strives to improve the flexibility and application of value assessment methods and models. IVI’s work is delivered in an open-source environment, providing transparency to its users.\textsuperscript{74} IVI states that its work is intended to support improved health care decision making, serving as a laboratory for testing new methods to improve value assessment. Thus far, IVI is unique in having developed alternative, disease-specific value models for rheumatoid arthritis, non–small cell lung cancer, and major depressive disorder. IVI’s leadership has stated publicly, “Structural deficiencies in the models that underlie value assessment have perpetuated health inequities. Communities of color and other groups are generally not represented in the data used to make health care decisions, routinely disadvantaging them. Yet this broken framework is what value assessment continues to be built on.”\textsuperscript{75} As described earlier in this brief, IVI does use Multi-Criteria Decision Analysis, which utilizes the QALY as an output.
iii. The Patient-Driven Values in Healthcare Evaluation (PAVE) Center

The Patient-Driven Values in Healthcare Evaluation (PAVE) Center is a Center of Excellence in Patient-Driven Value Assessment at the University of Maryland dedicated to promoting “value-based decision-making through a diverse multistakeholder collaboration and engaging patients from an extensive network of partners to build technical expertise in patient-centered outcomes research, education, and dissemination.” Its mission includes developing approaches to value assessment that solicit meaningful input from patients, ensure transparency in methods and decisions, and develop creative solutions to meet the needs of all health care decision makers. For example, the PAVE Center is working to advance a patient-perspective approach to guide value assessments of COVID-19 vaccines, hepatitis C treatment, and depression (in partnership with IVI).

iv. National Health Council (NHC)

National Health Council (NHC) is a nonprofit organization made up of over 140 national health-related organizations and businesses, including the nation’s leading patient organizations. In 2018, NHC convened representatives of organizations producing value frameworks and/or assessments with participants from patient groups that have interacted with those organizations to articulate a shared vision for what marks success in enhanced patient centricity in value assessment. NHC identified recommendations for gathering patient input through meaningful patient engagement in a manner that is incorporated into the design, conduct, and translation of real-world research reflecting patients’ lived experiences. NHC has created a “Value Classroom” with support from PCORI to help patients access basic information about health economics, value assessment terminology, and information to help patients and organizations engage in the value assessment process.
Recommendations

**U.S. Department of Health and Human Services (HHS) Center for Medicare and Medicaid Innovation (CMMI)**

CMMI should study alternatives to standard CEA that assess the value of drugs and other health interventions such as the Efficiency Frontier, Burden Augmented by Deadliness and Impact, Health Years in Total, Multi-Criteria Decision Analysis, and the Generalized Risk-Adjusted Cost-Effectiveness framework. To the extent that a method utilizes the QALY, the examination should determine whether these methods can be applied in a nondiscriminatory manner.

CMMI should implement a formal shared decision making program for both Medicare and Medicaid that applies the shared decision making standards and patient decision aids that have been developed through its grant-funded projects under the ACA.

**Patient-Centered Outcomes Research Institute (PCORI)**

PCORI should fund research that further develops and tests alternative methods and frameworks for determining the value of prescription drugs and other health interventions, that is, the Efficiency Frontier; Burden Augmented by Deadliness and Impact; Health Years in Total, Multi-Criteria Decision Analysis, and the Generalized Risk-Adjusted Cost-Effectiveness framework. The studies should focus on developing utilities/weights needed to use with the alternatives because this is the most needed research in this area. To the extent that a method utilizes the QALY, the examination should determine whether these methods can be applied in a nondiscriminatory manner.
**Agency for Healthcare Research and Quality (AHRQ)**

The Agency for Healthcare Research and Quality (AHRQ) should fund research to continue the development and testing of alternative methods to standard CEA for assessing the value of prescription drugs and other health interventions, that is, the Efficiency Frontier; Burden Augmented by Deadliness and Impact; Health Years in Total, Multi-Criteria Decision Analysis, and the Generalized Risk-Adjusted Cost-Effectiveness framework. The studies should focus on developing the weights needed to use with the alternatives because this is the most needed research in this area. To the extent that a method utilizes the QALY, the examination should determine whether these methods can be applied in a nondiscriminatory manner.

**The Institute for Clinical and Economic Review (ICER)**

ICER should continue to examine and empirically pilot innovative approaches that do not rely on the QALY, and further develop its existing engagement with patient groups to inform its reports with patient perspectives on value.
Endnotes

3 Ibid.
8 Technical review comment of Dr. Steve Pearson of the Institute of Clinical and Economic Review.
10 Basu, Anirban, one of the researchers who developed the HYT methodology: “It is actually advantageous to keep the calculations of the units of health produced separate from how that should be valued in different subgroups. For example, it would be important to establish that new treatments produce the same incremental life years in patients with diabetes and also patients with metastatic cancer. But distributional methods can put more weight on the gains for the latter group as they may have greater Life Year shortfall.” Comment made August 2022.
11 See endnote 4.
13 See endnote 6.
14 Technical review comment of Dr. Jaime Caro.
18 See endnote 1.


Ibid.


Interview with Dr. Jaime Caro, November 12, 2021.


Id. at endnote 38.


45 Ibid.
46 Ibid.
51 See endnote 50.
52 Technical review comment of Charles Phelps, August 2022.
54 “Who We Are.” Innovation and Value Initiative. https://www.thevalueinitiative.org/who-we-are/.
57 Ibid.
62 Preference-sensitive conditions are medical conditions for which the clinical evidence may not clearly support one treatment option, and the appropriate course of treatment depends on the values or preferences of the beneficiary regarding the benefits, harms, and scientific evidence for each treatment option.


Social Security Act, U.S. Code 42 S. 1181 (1320) (e).


