

**Defining Value – *For Us* or *With Us*:
Moving Us To Patient Oriented Value**

Haystack Project

Agenda

- Background
- Introduction to Value Frameworks
- QALY and ICER Patient Group Responses to ICER Assessments
- Patient-Centered Value Initiatives
- Next Steps

Five Primary Value Frameworks

Patient-Centric Value Frameworks Under Development



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Five Primary Value Frameworks: Brief Descriptions

Framework	Intended Purpose	Primary Treatment Focus	Primary Output
ICER	Develop a conceptual framework to help inform users, primarily insurers, in their assessments of the value of medical services, including drugs, medical devices, and procedures	Drugs / biologics; extended to devices, procedures	Long term care value (cost effectiveness); short-term budget impact; value-based price benchmarks
ACC-AHA	Provide a more complete examination of cardiovascular care, helping to generate the best possible outcomes within the context of finite resources	Cardiovascular treatments, primarily drugs	Three value levels: high value, intermediate value, low value – levels correspond to cost effectiveness thresholds
ASCO	Enable a physician and patient to assess the value of a particular cancer treatment regimen given the patient's individual preferences and circumstances	Cancer drug / biologic regimens	Net Health Benefit (NHB) comprised of clinical score, toxicity score, and bonus points for symptom palliation, treatment-free survival, QoL; average sales cost of drug per month
DrugAbacus	Provide an interactive tool to help determine the price of a cancer drug based on its value compared with the price assigned by the pharmaceutical company	Cancer drugs / biologics	Abacus price/month based on efficacy, tolerability, novelty, R&D costs, rarity, population burden, unmet need, and prognosis, as well as user preferences
NCCN	Provide the health care provider and the patient information to make informed choices when selecting systemic therapies based upon measures related to treatment, supporting data, and cost	Treatment regimens, primarily cancer drugs / biologics and non-pharmacologic modalities	Evidence Blocks for efficacy, safety, quality of evidence, consistency of evidence, affordability on scales of 1-5

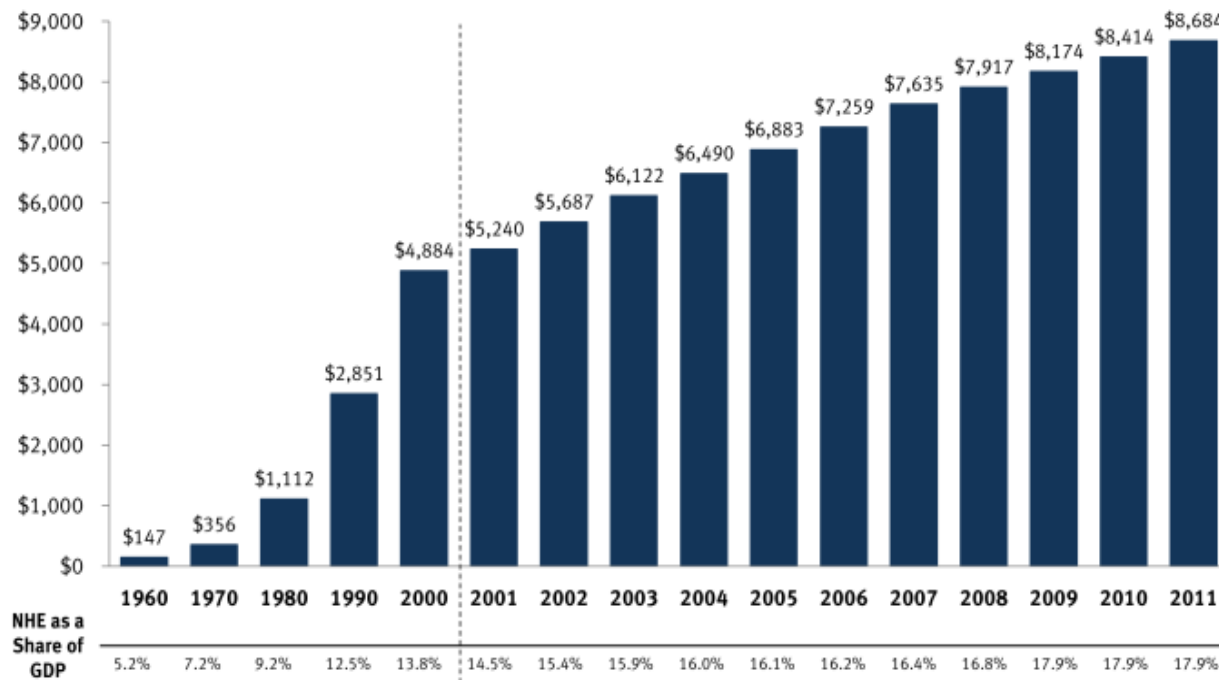
ICER focuses on producing value assessments of new drugs – sometimes even before FDA approval

<p>Audience</p>	<ul style="list-style-type: none"> ✓ Primarily payers, also patients and doctors ✓ Covers all treatment types, but recently said all rare and ultra rare (due to high cost) ✓ Has evaluated rare cancer treatments so early in life cycle -- ASH opted not to contribute input ✓ Medicare has cited to and quoted from ICER ✓ Claims to encourage stakeholder input, proactively engages stakeholders it identifies as interested, including patient groups
<p>Methods</p>	<p>Comparative effectiveness, long term value for the money, incremental cost effectiveness, other benefits and disadvantages, contextual considerations, short-term budget impact i.e., nothing that captures the value of a new treatment for patients w/ rare diseases with no current treatments</p>
<p>Rigor</p>	<ul style="list-style-type: none"> ✓ Claim to use variety of evidence/best practices for their cost-effectiveness analyses, incl. formal mathematical models with sensitivity analysis and comparison of costs per QALYs; ✓ But everything is weighted to get to the “right” answer – i.e., more weight to evidence that is blinded, randomized, placebo-controlled, large study population – all factors that inevitably disfavor rare disease treatments; ✓ In reality, for the end user, it all comes down to the QALY...

*From December 2014 to November 2018, ICER has convened **27 committees to review approximately 150 treatments. Of these, 15 treatments were for rare diseases and 4 for ultra-rare diseases (if defined by a prevalence of < 20,000 in the US)***

In response to rising healthcare costs, payers are turning to value assessment frameworks to design sustainable budgets

National Health Expenditures per Capita



- In response to rising healthcare costs, the prices of many treatments have garnered increased scrutiny
- Due to these treatments ranging widely in cost and yielding variable health outcomes, there has been a movement to determine the “value-based” price for treatments to support the healthcare system's shift from volume to value
- Nonprofit organizations, professional associations, and healthcare institutions have begun to assess more closely the real and perceived “value” of high-cost therapies through the development of value assessment frameworks

NOTE: According to CMS, population is the U.S. Bureau of the Census resident-based population, less armed forces overseas and their dependents.

SOURCE: Kaiser Family Foundation calculations using NHE data from Centers for Medicare and Medicaid Services, Office of the Actuary, National Health Statistics Group, at <http://www.cms.hhs.gov/NationalHealthExpendData/> (see National Health Expenditures by type of service and source of funds; file nhe11.zip); Gross Domestic Product data from Bureau of Economic Analysis, at <http://bea.gov/national/index.htm#gdp> (file gdp1ev.xls).

Payers Appear Poised to Re-think How to Decide Who Gets Access to New Ultra Rare and Rare Cancer Treatments Based on “Value”

Conventional Wisdom...

- Public and private payers will cover on-label use of new drugs.
- Cancer drugs are covered on-label and for off-label use supported by evidence.

Looking Ahead...

- Payers are questioning sustainability of health care financing due to finite resources and rapid evolution of treatment innovations, particularly in oncology.
- Value-based drug pricing/outcomes-based contracting appear to be emerging as reasonable compromise to balance costs and access.
- However, cost-effectiveness, comparative effectiveness, and “long-term value for the money” analyses are making their way into the debate.
- Example: Medicare proposes, for the first time, to restrict conditions for on-label and compendia-listed uses of an entire new class of treatments FDA-approved for rare lymphoma and leukemia now, and in clinical trials to treat other rare cancers in the future.

Treatments for Life-Threatening, Rare Diseases Are at a Disadvantage in Value Assessment Frameworks

Value Assessments are Supposed to be Evidence-Based;
“Strength of Evidence” is Key Determinant of Value

Disease Rarity

Small studies inevitable in small populations; viewed as weak evidence in value frameworks
Value frameworks seek data on treatment use in patient subpopulations (e.g., elderly)

Life-Threatening Disease

Lack of alternative treatment options make a “standard of care” control impossible, and placebo-controls are unethical for life-threatening diseases like cancer

Single arm studies are consistently discounted as suboptimal for value-assessment purposes and yet single arm studies are becoming the norm for rare/ultra rare.

Public Interest In Rapid
Availability

Cancer treatments for conditions without other options get FDA approved based on surrogate outcomes like complete response, without long-term outcomes data

Value frameworks seek robust data, long-term follow-up since “value” is based on added years

“Value frameworks” evaluate and quantify benefits, harms, and often costs – to get a *COMPOSITE VALUE METRIC*

IN GENERAL:

- ✓ Intended to inform all stakeholders across the care continuum of a treatment’s cost versus benefit
- ✓ Usually rely on OBJECTIVE outcomes supported by ROBUST data
- ✓ May result in a calculation in terms of dollars per ‘health unit’ gained
- ✓ ***Most target a payer audience, rather than physicians or patients***
- ✓ Payers in markets outside the US may use these assessments to restrict access, or even decline to cover at all unless the manufacturer reduces the price to reflect calculated value
- ✓ Not new concept in the US or other countries, but US payers historically have avoided constricting access to cancer treatments based on the cost of each year of life gained
- ✓ Public entities in other countries produce Health Technology Assessments (HTAs) (e.g., NICE in the UK), which are essentially what we call value frameworks in the US
- ✓ US payers seem to be inching toward incorporating cost-effectiveness or value into access decisions **EVEN WHEN THERE IS NO OTHER FDA APPROVED TREATMENT**

Value Frameworks were created to assist with formulary and patient-physician decision making

Value frameworks are ill suited to incorporate the patient experience and other nonclinical factors

Pros

- Intent is to improve decisions for payers, providers, and patients
- Intent is to encourage use of treatments that produce better health
- Enhance dialogue on value of care for the cost/price paid
- May improve medical decisions while reducing costs IF subject treatments are compared to other medically-accepted therapies for the disease, but function as gatekeeper to medical care if applied to rare, life-threatening diseases without adequate alternative treatments

Cons

- Based on averages – by definition, has to ignore clinical nuances and individual patient factor and preferences (e.g., some patients willing to accept more safety risks to improve efficacy while others may prefer treatments with less safety risk even if efficacy is reduced; i.e., Element of Value/previous slide)
- No clear “best practices” or measures of value (e.g., assigning different values to improved health outcomes, defining value based on benefit vs. cost of care)
- Controversial when applied to rare diseases
- Limited definition of “productivity” in economic terms negates potential productivity loss in retired persons
- Limited patient input in framework conception and development – may be missing elements important to patients
- Ethical concerns regarding care rationing – complex and controversial

“Core” elements are incorporated into value calculation;
“Novel” elements are usually relegated to context.

Elements of value	Type of element	Features of medical technologies in which element is relevant in value assessment
Net costs	Core	Can apply to all assessments
QALYs gained	Core	Can apply to all assessments
Productivity	Novel	Relevant when treatment has an impact on productivity
Adherence-Improving factors	Novel	Relevant when features of the treatment itself improve adherence with the treatment
Reduction of uncertainty due to new diagnostic	Novel	Relevant when the treatment is accompanied by a companion diagnostic test
Caregiver burden	Novel	Relevant when caregivers need to assist patient with activities of daily living / medical tasks
Insurance value	Novel	Relevant when baseline health status is particularly poor
Severity of disease	Novel	Relevant when considering treatments for end-of-life care and/or high-severity conditions
Value of hope	Novel	Relevant when therapies have uncertain effects that cannot be predicted by diagnostic test
Real option value	Novel	Relevant when technology extends life of patient
Equity	Novel	All
Level of Innovation	Novel	Relevant when technology identifies a new mechanism of action, treatment approach, or mode of delivery

The QALY was initially **developed in the UK in the 1970s** as healthcare expenditures dramatically increased

Background on History of QALY

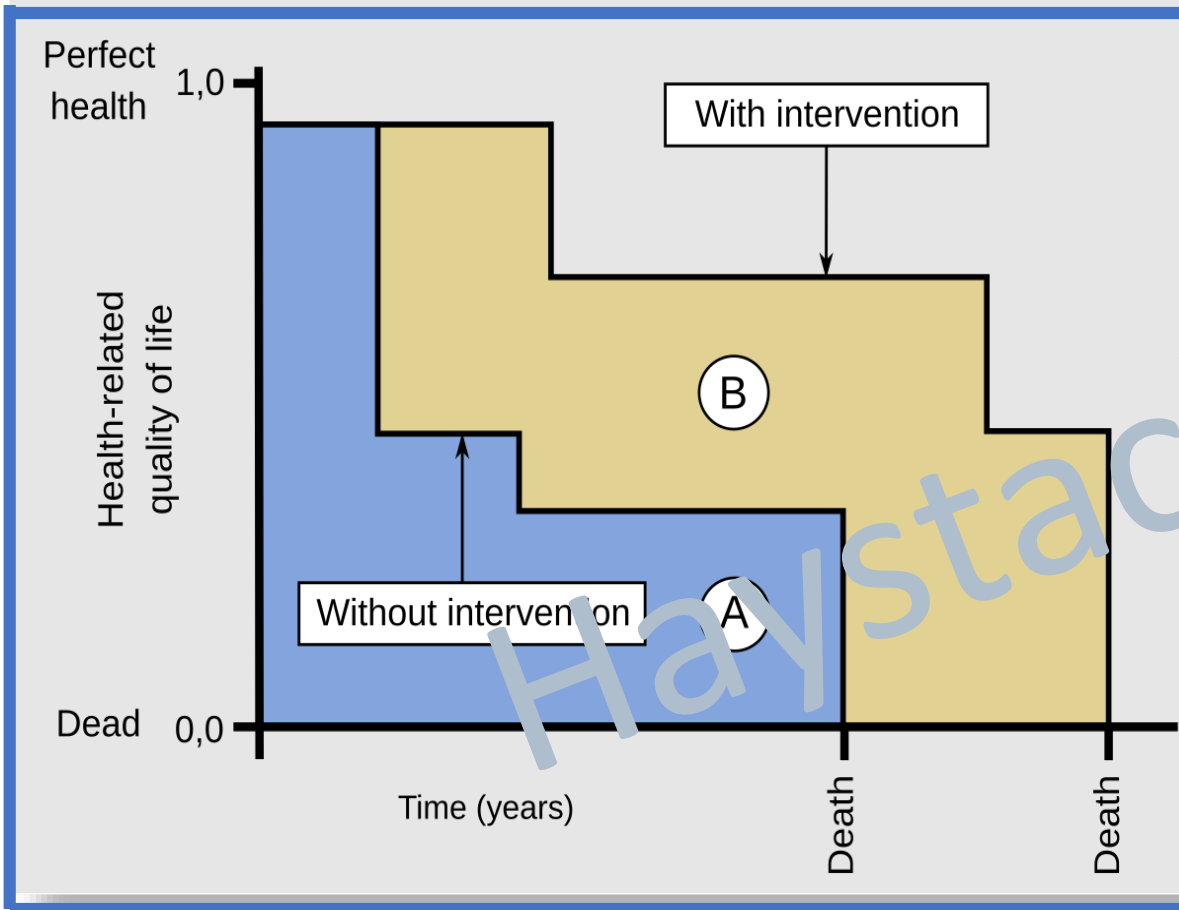
- Popularized in the 1970s in the UK in response to a need for improved decision-making around healthcare expenditures
- The measure was first developed by the **National Institute for Health and Care Excellence (NICE) in the UK**, which conducts technology appraisals using the QALY measurement
- NICE uses a 'standard' threshold of **\$26,500 - \$40,000 per QALY** when appraising technologies
- More recently, in April 2017, NICE adopted a **higher threshold of between \$133,000 and \$397,000 per QALY** when appraising treatments *for 'very rare diseases'*

NICE National Institute for
Health and Care Excellence

Founded in 1999

The foundation of cost-effectiveness assessments is the Quality Adjusted Life Year (QALY)

What is a quality-adjusted life-year (QALY)?



Years of Life x Utility Value = #QALYs

- Measures improvement in **length** and **quality** of a patient's life
- Often uses **individual and community (not patient) preferences** for different health conditions ("health states")
- **Compares interventions** that are relatively inexpensive (low cost per QALY) to those that are relatively expensive (high cost per QALY)
- Is QALY a useful metric for evaluating value of **potentially curative treatment for patients with high mortality risk?**
- QALYs may consider side effect profile, but risk/benefit analysis **rarely considers patient preferences**

Measuring patient outcomes (“utility values”) in objective, quantifiable terms as “value” is challenging *AND SQUISHY*: different methodologies, inputs, and timelines yield different results

Direct Methods

- Individuals are asked to describe and assess health states and place weights

Indirect Methods

- Individuals are asked to fill out a preference-based questionnaire (E.g., EuroQol-5D, SF-6D)

Point of disagreement in measuring value: Who’s perspective should be used?

Patients living with the disease?

Members of the general public?

People who have first-hand experience with outcomes?

People with no experience?

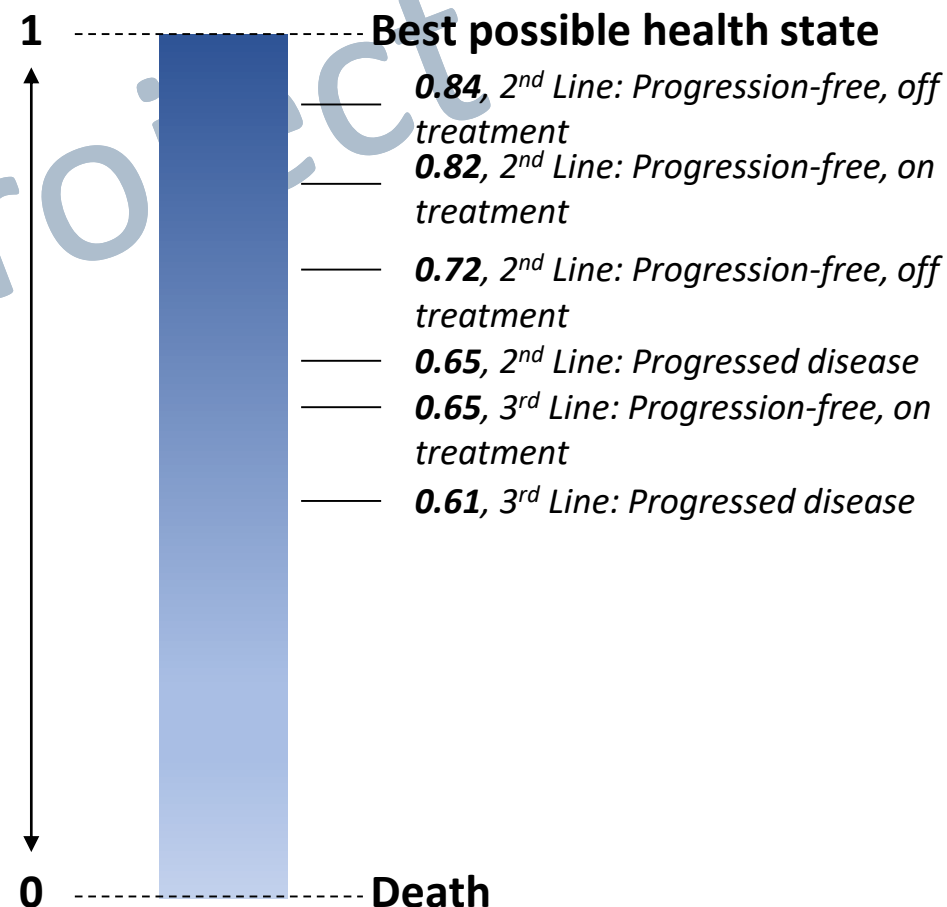
Caregivers?

Different health conditions get assigned different patient outcomes on a scale of 1 to 0 which are then multiplied by life years gained to yield the number of QALYs

Utility Values Background

- Health state preference values are part of the equation in which a person's quality of life is estimated
- The utilities that are produced represent the valuations attached to each health state on a continuum between 0 and 1, where 0 is equivalent to being dead and 1 represents the best possible health state -
- Sample health states include:
 - Progression free, off treatment
 - Progression free, on treatment

Example Utility Values (from ICER Multiple Myeloma Assessment)



Example Quality-Adjusted Life Year (QALY) calculation

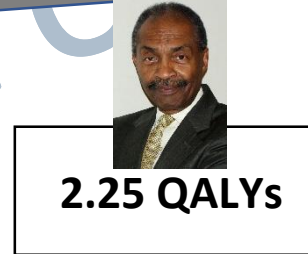
Quantity of Life

Quality of Life

QALYs



Standard of care



New drug



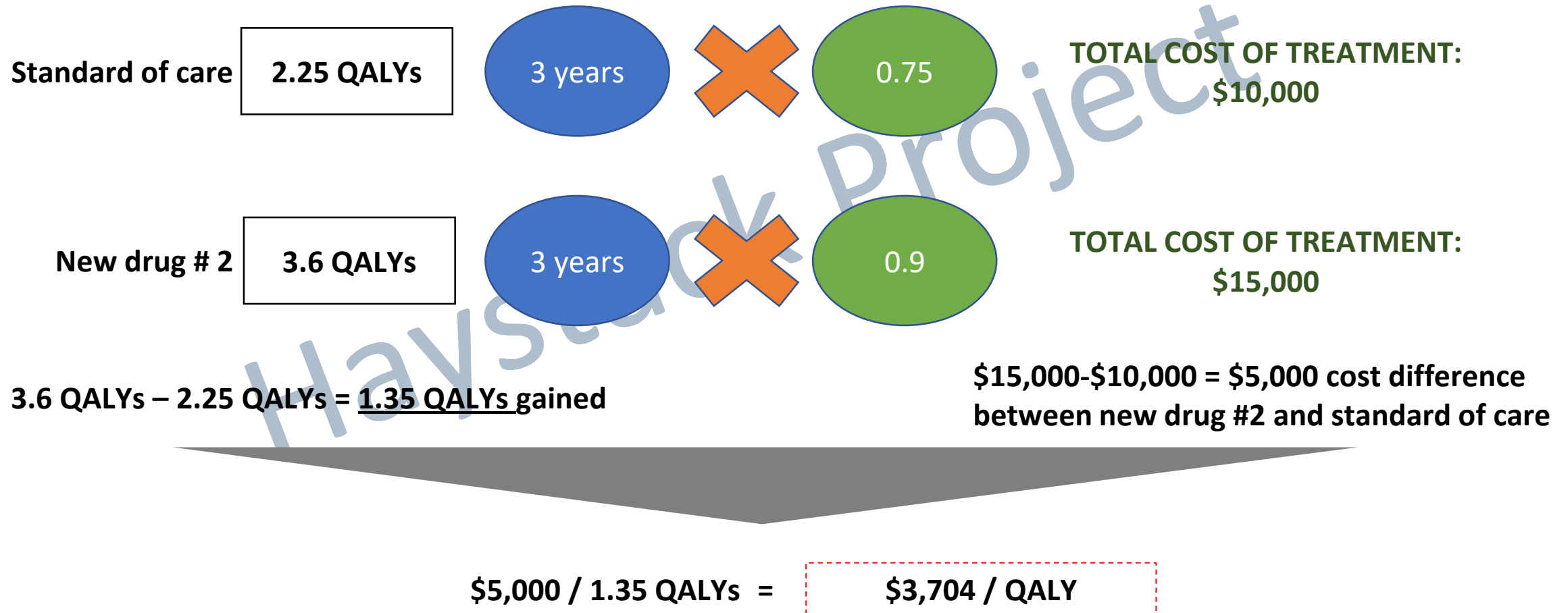
New drug results in .30 QALYs vs. standard of care

New drug # 2



New drug results in 1.35 QALYs vs. standard of care

The incremental cost-effectiveness ratio is used to compare the “efficiency” of health interventions



QALYs are not patient centric and do not incorporate individual treatment goals

Limitations of the QALY

Measurement

- While some health economists try to justify these values through laborious studies that compare the costs of various medical services, the threshold amount is effectively random
- QOL measurement will depend on tool used and population

Heterogeneity

- Often considers only societal preferences, not patient preferences
- Do not take into account patient preferences around process of care (e.g. invasiveness of therapy)
- Does not reflect differences in patient characteristics – this is particularly important in rare

Neglects individual treatment goals

- Valuing “perfect health” over a pre-defined “less perfect health” (e.g. desiring a therapy because it may increase the chance of seeing one’s children grow to adulthood, enjoy an active retirement with a spouse, or even go on a long-awaited vacation)

Devalues disability and age

- Elderly and disabled disadvantaged, who cannot achieve maximum QALY scores because they will never achieve the highest “quality of life”

Ill-suited for rare disease drugs

- New therapies for rare diseases are, approved with fewer subjects in clinical trials, and predicting the longevity of patients in these trials is difficult
- Prices of these drugs tend to be higher, and in rare and ultra-rare cancer, trials often lack long-term data. Note: waiting for data to support “value” could delay access for 2, 5, or more years

A recent study found that **only 25% of patients believed QALY was a good way to measure value** in healthcare and that the **QALY does not reflect the diversity of their experiences, needs, and beliefs**¹

Both private and public payers are increasingly looking to value frameworks like ICER to manage pharmaceutical spending



In 2018, CVS Health announced a new formulary management option that allowed self-insured employers to remove from their formularies medicines launched at a price greater than \$100,000 per quality-adjusted life-year (QALY)



New York's Drug Utilization Review (DUR) Board is permitted to use QALY-based standard to determine medicine reimbursement and coverage policies; the the most recently released Executive Budget proposal would make permanent the use of cost-effectiveness assessments conducted by third-parties



In 2017, ICER announced that it was collaborating with the Department of Veterans Affairs (VA) Pharmacy Benefits Management (PBM) Services to “support VA coverage and price negotiations with pharmaceutical companies to promote access to high-value drugs”

Five Primary Value Frameworks




Patient-Centric Value Frameworks Under Development



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Recently developed patient-centered value frameworks have made attempts to capture factors that are relevant and important to patients

Patient Centric Value Frameworks

	Year of Development	Objective
	2016	<p>Provide a tool that the patient community, physicians, health systems, and payers can use to evaluate the patient centeredness of value models and to guide value model developers on the meaningful incorporation of patient engagement throughout their processes.</p>
	2017	<p>Provide high-level methodology for how to assess the value of multiple health care options from the patient’s perspective. It is made up of a set of patient-centered domains, technical criteria, and measures. It includes a methodology and specified types of data that can be applied in a variety of ways with additional analyses</p>
	2018, 2019	<ul style="list-style-type: none"> • Build an open-source platform that facilitates robust and rigorous patient-centered value assessment of health technologies tailored to the needs and interests of individual decision makers. • So far only developed for Rheumatoid Arthritis and Non-Small Cell Lung Cancer

Manufacturers and patient groups are invited to comment on ICER assessments

Comment letters issued by manufacturers and patient groups for ICER assessments on cancer therapies have cited common concerns:

AMGEN

April 15, 2016; Submitted Electronically to publiccomments@icer-review.org from Martin Zagari, MD, Vice President, Global Health Economics, Amgen on behalf of Amgen

Re: ICER

Dear Dr. P:

At Amgen, our mission is to improve the lives of patients with cancer through innovative treatments. We are committed to providing the highest quality of care and/or research that will improve the lives of patients with cancer. We are committed to providing the highest quality of care and/or research that will improve the lives of patients with cancer.

Recent Focus Areas: Improving the lives of patients with cancer through innovative treatments. We are committed to providing the highest quality of care and/or research that will improve the lives of patients with cancer.

The National Cancer Institute (NCI) is the lead agency for the National Cancer Program. The NCI is committed to providing the highest quality of care and/or research that will improve the lives of patients with cancer.

After careful review of the ICER report, we conclude that the ICER report is flawed in several key areas:

1. Perform an evaluation of the evidence base. a. b.
2. Used outdated models.
3. Ignored outcomes.
4. Provide value estimates.
5. Overestimated costs.
6. Failed to consider the impact of the ICER's analysis. We supplement

CANCER SUPPORT COMMUNITY
A Global Network of Education and Hope

April 21, 2016

Steven D. Pearson, MD, MSc, FRCP
President, Institute for Clinical and Economic Review
Boston, MA 02109 USA

RE: Institute for Clinical and Economic Review (ICER) Assessment of Drug Therapies for Multiple Myeloma

Dear Dr. Pearson,

On behalf of the Cancer Support Community, an international nonprofit organization that provides support, education and hope to more than one million people affected by cancer each year, we appreciate the opportunity to respond to the request for comments regarding ICER's draft report of treatment options for relapsed or refractory multiple myeloma.

As the largest direct provider of social and emotional support services for people impacted by cancer, and the largest nonprofit employer of oncology mental health professionals in the United States, CSC has a unique understanding of the cancer patient experience. Each year, CSC serves people affected by cancer through its network of 44 licensed affiliates - more than 120 satellite locations, a toll free telephone hotline and a vibrant online community - and delivers more than \$40 million in free, personalized services each year.

Additionally, CSC is home to the Research and Training Institute - the only entity of its kind focused solely on the cancer patient experience. The Research and Training Institute has contributed to the evidence base regarding the cancer patient experience through its Cancer Experience Registry®, various publications and peer-reviewed studies on distress screening, and the psychosocial impact of cancer and cancer survivorship, to name a few. This combination of direct services and research uniquely positions CSC to provide organizations like ICER with feedback based on evidence as well as real world impact.

CSC acknowledges ICER's intent to seek multi-stakeholder input as a part of the process involved in assessing the value and effectiveness of different oncology treatment regimens. However, we encourage you to seek input earlier in the process and in a more comprehensive way. Both the conversation on value and multi-stakeholder engagement are at the core of CSC's work on access, and we are eager to work with you to move appropriate solutions forward.

Dr. Pearson, we are concerned about several items, which I have outlined below.

Lack of consideration of the patient definition of value

At the Cancer Support Community, the patient experience drives all aspects of our work: direct service delivery, research and policy. To better understand the needs of people with cancer, we developed the Cancer Experience Registry. The Registry collects survey data that measures all aspects of the cancer experience including, the social, emotional, spiritual and financial effects of cancer on the person diagnosed as well as his or her family. Over 9,300 people have participated so far, sharing almost one million data points about their experience.

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1050 17th Street NW, Suite 900, Washington DC 20006
202.699.9709 Phone 202.974.7999 Fax 888.793.9355 Toll Free

New York City Office:
163 West 46th Street, Suite 1002, New York, NY 10036
917.305.1200 Phone 212.967.8717 Fax 888.445.3248 Toll Free

Research & Training Institute:
4150 Chancery Drive, Fairmount Park, Philadelphia, PA 19131
267.295.3000 Phone 215.883.2580 Fax

- Ignoring patient experience by not appropriately valuing health-related quality of life outcomes
- Neglecting diversity of certain disease population (i.e. “one-size-fits-all” approach)
- Defining “productivity” in economic terms rather than from patient perspective
- Over-estimating down side of short term side effects
- Downgrading strength of evidence when pivotal trials were small and/or did not have blinded, randomized, assignment to standard of care or drug
- Lack of adequate standard care for comparison
- Outcomes selected by ICER are not clinically meaningful or reflective of patient preferences
- Lack of transparency surrounding methodology and calculations

Case Study: National Multiple Sclerosis Society (NMSS)

- **Patient Organization:** NMSS is part of the MS Coalition
- **Value Assessment:**
 - ICER Assessment on: *Therapies for Relapsing-Remitting and Primary-Progressive Multiple Sclerosis: Effectiveness and Value* (publicly released March 6, 2017)
- **What NMSS did:**
 - Commented at every official opportunity and had informal conversations
 - With the MS Coalition, created an online questionnaire to assess patient perspectives on the most important issues for patients when making decisions about which therapy to take
- **Impact:**
 - Patient perspectives were included in the narrative of the report; voting committee members cited patient considerations were top of mind when voting
 - However, patient perspective was **not incorporated in cost per QALY calculation**

Case Study: Parent Project Muscular Dystrophy (PPMD)

- **Patient Organization:** Patent Project Muscular Dystrophy (PPMD)
- **Value Assessment:**
 - ICER Assessment on: *Assessing the Effectiveness and Value of Drugs for Rare Conditions (May 2017)*
- **What PPMD did:**
 - Became familiar with ICER review process and framework
 - Contacted ICER to understand how to assist during process & report, whether PFDD data and rare disease considerations would factor into review— initially phone call was not returned!
 - Provided ICER with rare disease community experts and commented at each opportunity
 - Helped plan ICER Orphan Drug Assessment & Pricing Summit by serving on Working Group that informed Summit agenda and briefing paper
- **Impact:**
 - Comment period remains open – Impact still unknown. Relationship established & awareness of complexities of reviews in rare disease heightened
 - However, patient perspective was **not incorporated in cost per QALY calculation**

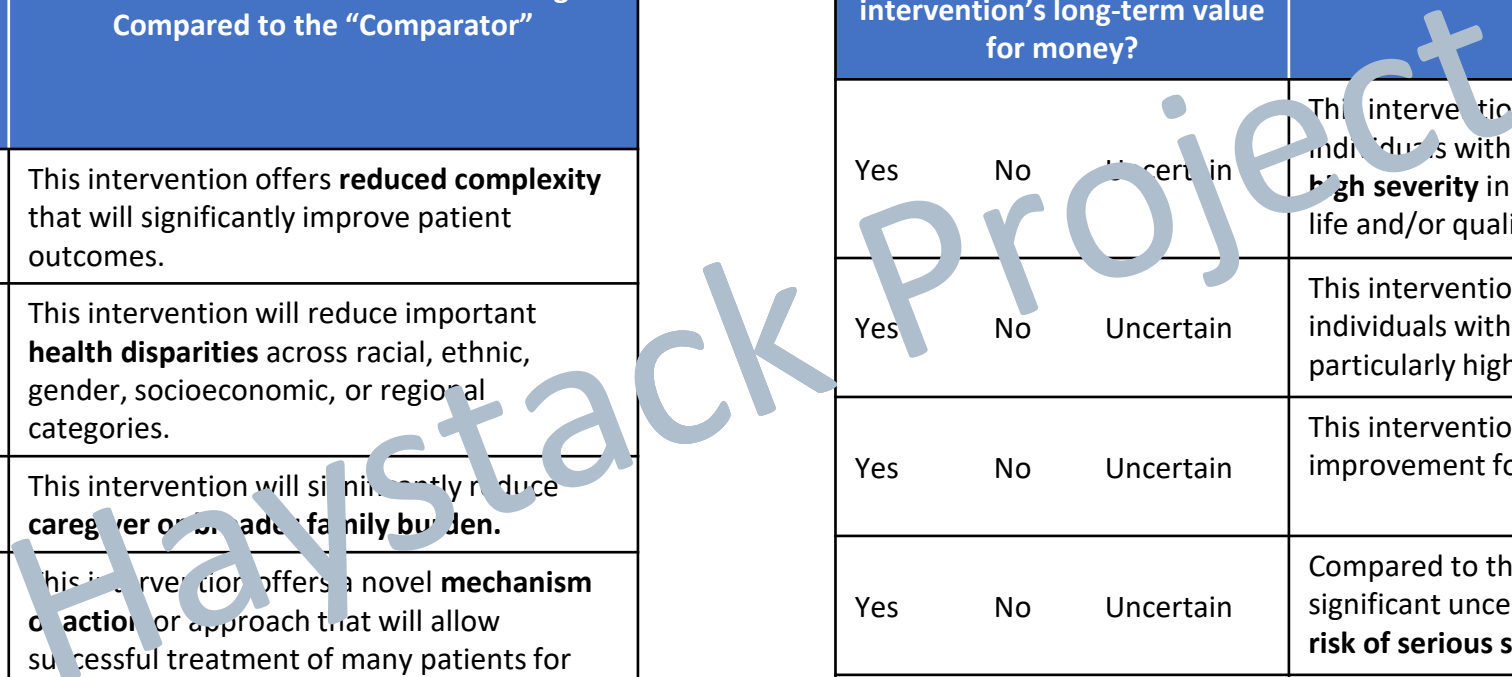
ICER is piloting including other “benefits and disadvantages” and “contextual considerations” in reports, outside the final ratings payers use

Other Benefits and Disadvantages

When compared to the comparator used in the cost-effectiveness analysis, does this particular intervention offer one or more of the following “other benefits or disadvantages?”	Potential Other Benefits or Disadvantages: Compared to the “Comparator”
Yes No Uncertain	This intervention offers reduced complexity that will significantly improve patient outcomes.
Yes No Uncertain	This intervention will reduce important health disparities across racial, ethnic, gender, socioeconomic, or regional categories.
Yes No Uncertain	This intervention will significantly reduce caregiver or caregiver family burden.
Yes No Uncertain	This intervention offers a novel mechanism of action or approach that will allow successful treatment of many patients for whom other available treatments have failed.
Yes No Uncertain	This intervention will have a significant impact on improving return to work and/or overall productivity
Yes No Uncertain	There are other important benefits or disadvantages that should have an important role in judgements of the value of this intervention

Contextual Considerations

Are any of the following contextual considerations <u>important</u> in assessing this intervention’s long-term value for money?	Contextual Considerations
Yes No Uncertain	This intervention is intended for the care of individuals with a condition of particularly high severity in terms of impact on length of life and/or quality of life.
Yes No Uncertain	This intervention is intended for the care of individuals with a condition that represents a particularly high lifetime burden of illness
Yes No Uncertain	This intervention is the first to offer any improvement for patients with this condition.
Yes No Uncertain	Compared to the “comparator,” there is significant uncertainty about the long-term risk of serious side effects of this intervention
Yes No Uncertain	Compared to “the comparator,” there is significant uncertainty about the magnitude or durability of the long-term benefits of this intervention
Yes No Uncertain	There are additional contextual considerations that should have an important role in judgements of the value of this intervention: _____



FDA launched the patient-focused drug development (PFDD) initiative (2013) to incorporate the patient voice in drug development and evaluation

The PFDD initiative aims to:

- **Facilitate and advance use of systematic approaches** to collecting and utilizing robust and meaningful **patient and caregiver input** to more consistently inform drug development and regulatory decisions
- **Encourage identification and use of approaches and best practices** to facilitate enrollment and minimize burden of participation in clinical trials
- **Enhance understanding and appropriate use of methods** to capture information on **patient preferences** & tradeoffs between treatment benefit and risk
- Identify the information most important to patients on **treatment benefits, risks, and burden, and how to communicate the information** to support decisions

FDA activities related to this initiative include:

- Conducting **24 disease-specific meetings** to more systematically gather patients' perspectives on their condition and available therapies to treat their condition and generated **Voice of the Patient** reports, which summarize the input provided by patients and patient representatives at each of these public meetings
- Developing a series of methodological guidance on **the collection of patient experience data**, and the **use of such data and related information in drug development**
- Developing guidance on **developing and submitting proposed draft guidance relating to patient experience data**

Discussion Questions...

1. Do patient groups have a list of interventions that may be evaluated by any of these Value Framework developers?
2. Do you keep track of what has been approved by the FDA or in the pipeline?
3. Have you ever asked your patients/caregivers to describe the value of each intervention?
4. What improvements over current standard of care are most important?
5. Are there unique factors associated with the cancer population that should be considered?
6. Once ICER decides to evaluate a treatment, comment periods are on very short timelines (generally 3 weeks or less) – How quickly can your organization compile a compelling response and enlist other stakeholders to join your efforts?

Next Steps...

1. Would you be interested in making a list of interventions that may be evaluated by a Value Framework developer? Would you know how? Would you need help?
2. Would you be interested in surveying your patients – directly or indirectly – to describe the value of a coming intervention? Would you know how? Would you need help?
3. Do you see value in participating in ICER's process or in issuing your own "Voice of the Patient" type reports? Do you see independent value in these?

BACK UP SLIDES

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Questions Every Legislator Should Ask About ICER: Ethical Considerations

- Is it ethical to deny patients a new therapy pending an ICER review?
- Do QALY-based reviews capture the real-world experiences of patients with particular therapies?
- Do QALY standards discriminate against the disabled by assigning a lower quality of life score for disabilities?
- In a related question, does the QALY standard discriminate against older Americans by denying them palliative care?
- Is the use of ICER reviews simply a method of dodging political accountability for rationing medicine?
- Wouldn't the use of ICER reviews drive profitability for private sector health plans and pharmacy benefit managers (PBMs), and represent a conflict of interest?
- Doesn't the QALY standard simply place an arbitrary value upon human life?
- Does the ICER review process interfere with autonomous physician-patient relationships?
- Is employing the ICER model a form of generational discrimination?

Questions Every Legislator Should Ask About ICER: Methodological Issues and Theoretical Assumptions

- Is the use of meta-analysis, i.e. the pooling of results from different studies with different assumptions and analyzing different targets, often using different methodologies, a sound way to reach conclusions about specific drug therapies?
- Are ICER reviews conducted with adequate data?
- Does QALY analysis lead to inefficiencies in spending in the healthcare system?
- Does QALY help legislators address budget challenges and shortfalls?
- Is ICER methodology overly quantitative and does it therefore fail to capture the variety of diverse circumstances that medical care presents?
- Should quality of life measurements be determined by patients or the general population?
- Should the ICER methodology be transparent?
- How often should ICER reviews be updated?
- Is the use of list prices in ICER reviews a serious methodological flaw?
- How long will ICER reviews take and will new drugs be available to patients pending the reviews?
- Is it arbitrary to establish a global budget for drug spending?
- Does the utilization of QALYs fail to capture the non-health benefits of drug therapies?
- Do QALYs discount the opinion of physicians in patient care?
- Does the ICER model discourage innovation?

Questions Every Legislator Should Ask About ICER: Condition-Specific Considerations

- Is the ICER model inadequate to evaluate orphan drugs and drugs for rare diseases such as gene therapies?
- Does the ICER model discriminate against preventative medicine?
- Will personalized medicine make the ICER model obsolete?
- Can the ICER model adequately capture the value of mental health treatments?
- Does the ICER model, like the NICE model, have an inherent bias against cancer treatments?
- Does the use of QALYs fail to capture the value of important nuances within specific disease areas?