

Value Assessments: The Impact on People with Rare Diseases

July 17, 2019

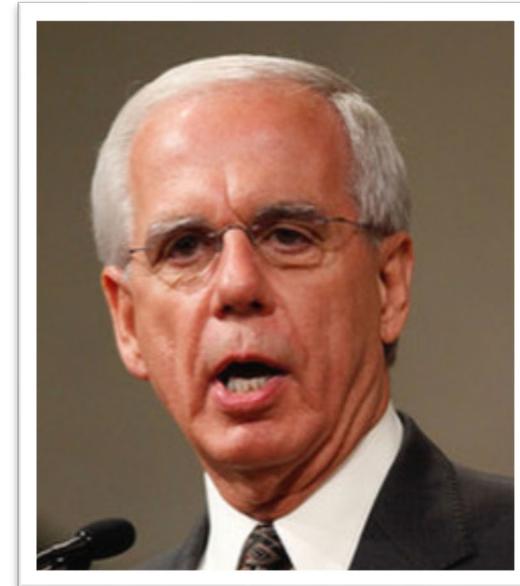


Partnership to Improve Patient Care: Background

- Coalition of organizations representing patients and people with disabilities, providers, researchers and innovators
- Support PCORI and PCORI Reauthorization
- Support patient-centeredness criteria and patient engagement as drivers of alternative payment models
- Support patient-centered models for the development of value frameworks and shared decision-making tools

Honorable Tony Coelho, PIPC Chairman

- Tony Coelho is a former United States Congressman from California and primary author and sponsor of the Americans with Disabilities Act
 - *“Nothing about us without us”*
- Tony is a person with epilepsy
- PIPC began as a coalition supporting PCORI’s creation, with a voice and a vote for patients and people with disabilities on its Board of Governors
 - *No person is average*

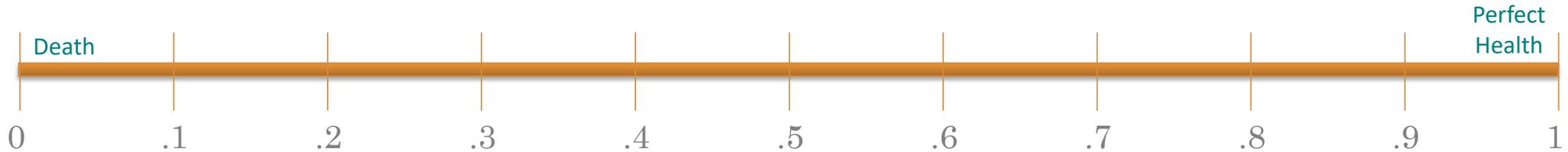


What is a QALY Exactly?



QALYs discriminate against people with disabilities by placing a lower value on their lives

What's the value of your life?



Person with Cancer



Person with Rheumatoid Arthritis



Person with Diabetes

QALYs as a Means of Measuring Health Quality

- Measure of disease or disability burden and treatment efficacy in mitigating it;
- 1 QALY = 1 year in 'perfect health',
- 0 QALY = Death
- $0 < x < 1$ = Disabled or sick life

Severe Parkinson disease	Parkinson disease, severe	difficulty in walking and daily activities. The person falls easily and has a lot of difficulty talking, swallowing, sleeping, and remembering things.	0.575 (0.396–0.73)
Severe epilepsy	Epilepsy, seizures >= once a month	has sudden seizures one or more times each month, with violent muscle contractions and stiffness, loss of consciousness, and loss of urine or bowel control. Between seizures the person has memory loss and difficulty concentrating.	0.552 (0.375–0.71)
Less severe epilepsy	Epilepsy, seizures 1-11 per year	has sudden seizures two to five times a year, with violent muscle contractions and stiffness, loss of consciousness, and loss of urine or bowel control.	0.263 (0.173–0.367)
Seizure-free, treated epilepsy	Generic uncomplicated disease: worry and daily medication	has a chronic disease that requires medication every day and causes some worry but minimal interference with daily activities.	0.049 (0.031–0.072)
Mild multiple sclerosis	Multiple sclerosis, mild	has mild loss of feeling in one hand, is a little unsteady while walking, has slight loss of vision in one eye, and often needs to urinate urgently.	0.183 (0.124–0.253)
Severe multiple sclerosis	Multiple sclerosis, severe	has slurred speech and difficulty swallowing. The person has weak arms and hands, very limited and stiff leg movement, has loss of vision in both eyes and cannot control urinating.	0.719 (0.534–0.858)
Moderate multiple sclerosis	Multiple sclerosis, moderate	needs help walking, has difficulty with writing and arm coordination, has loss of vision in one eye and cannot control urinating.	0.463 (0.313–0.613)
Asymptomatic multiple sclerosis	Asymptomatic	--	--
Severe respiratory problems and speech problems due to motor neuron disease	Severe respiratory and speech problems	(combined DW)	0.438 (0.304–0.581)
Moderate respiratory problems and speech problems due to motor neuron disease	Moderate respiratory and speech problems	(combined DW)	0.265 (0.184–0.36)
Mild respiratory problems and speech problems due to motor neuron			0.069

Symptomatic dysthymia	Major depressive disorder, mild episode	The person sometimes sleeps badly, feels tired, or has trouble concentrating but still manages to function in daily life with extra effort.	0.145 (0.099–0.209)
Bipolar disorder manic state	Bipolar disorder, manic episode	is hyperactive, hears and believes things that are not real, and engages in impulsive and aggressive behavior that endanger the person and others.	0.492 (0.341–0.646)
Bipolar disorder residual state	Bipolar disorder, residual state	has mild mood swings, irritability and some difficulty with daily activities.	0.032 (0.018–0.051)
Bipolar disorder depressive state	Major depressive disorder, moderate episode	has constant sadness and has lost interest in usual activities. The person has some difficulty in daily life, sleeps badly, has trouble concentrating, and sometimes thinks about harming himself (or herself).	0.396 (0.267–0.531)
Anxiety disorders, currently without symptoms	Asymptomatic	--	--
Moderate anxiety disorders	Anxiety disorders, moderate	feels anxious and worried, which makes it difficult to concentrate, remember things, and sleep. The person tires easily and finds it difficult to perform daily activities.	0.133 (0.091–0.186)
Severe anxiety disorders	Anxiety disorders, severe	constantly feels very anxious and worried, which makes it difficult to concentrate, remember things and sleep. The person has lost pleasure in life and thinks about suicide.	0.523 (0.362–0.677)
Mild anxiety disorders	Anxiety disorders, mild	feels mildly anxious and worried, which makes it slightly difficult to concentrate, remember things, and sleep. The person tires easily but is able to perform daily activities.	0.03 (0.018–0.046)
Anorexia nervosa	Anorexia nervosa	feels an overwhelming need to starve and exercises excessively to lose weight. The person is very thin, weak and anxious.	0.224 (0.15–0.312)
Bulimia nervosa	Bulimia nervosa	has uncontrolled overeating followed by guilt, starving, and vomiting to lose weight.	0.223 (0.149–0.311)

Severe cannabis dependence	Cannabis dependence	uses marijuana daily and has difficulty controlling the habit. The person sometimes has mood swings, anxiety and hallucinations, and has some difficulty in daily activities.	0.266 (0.178–0.364)
Other drug use disorders	Cocaine dependence, mild	uses cocaine at least once a week and has some difficulty controlling the habit. When not using, the person functions normally.	0.116 (0.074–0.165)
Severe major depressive disorder	Major depressive disorder, severe episode	has overwhelming, constant sadness and cannot function in daily life. The person sometimes loses touch with reality and wants to harm or kill himself (or herself).	0.658 (0.477–0.807)
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Peter Singer on “Why We Must Ration Health Care”

- “Disability advocates might argue that such judgments, made by people without disabilities, merely reflect the ignorance and prejudice of people without disabilities when they think about people with disabilities.”
- “We should, they will very reasonably say, ask quadriplegics themselves to evaluate life with quadriplegia. If we do that, and we find that quadriplegics would not give up even one year of life as a quadriplegic in order to have their disability cured, then the QALY method does not justify giving preference to procedures that extend the lives of people without disabilities over procedures that extend the lives of people with disabilities.”

Peter Singer on “Why We Must Ration Health Care”

- “This method of preserving our belief that everyone has an equal right to life is, however, a double-edged sword. If life with quadriplegia is as good as life without it, there is no health benefit to be gained by curing it.”
- Essentially, QALYs force a choice between access to specialized medical treatment and non-discrimination in health care.

Challenges with QALY Model

- Under population survey models, the non-disabled population may systematically overestimate the burden of life with disability.
 - Research suggests a majority of American public says they would rather have HIV than be blind (Scott, 2016).
- Common QALY measure (EuroQol-5D) rates inflammatory arthritis as “worse than death” (Harrison, 2009).
 - Significant variation between TTO and VAS quality of life assessments reported under EuroQol-5D
- Under models where PWD self-report QoL, well supported people with disabilities who report relatively high levels of quality of life due to access to adequate support may find it very hard to demonstrate sufficient gains in QoL due to treatment efficacy.

QALYs and evLYGs

The evLYG

ICER's future reports will incorporate more prominently a calculation of the Equal Value of Life Years Gained (evLYG), which 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.

Supplementing the QALY, Not Replacing It

To maintain the ability of cost-effectiveness analyses to reflect the full benefits that treatments may have on quality of life, ICER will continue to calculate each treatment's QALY gained. The cost per QALY gained remains the best way for policymakers to understand how well the price of a treatment lines up with its benefits and risks for patients.

By understanding a treatment's cost per evLYG, as well as its traditional cost per QALY, policymakers can take a broader view of cost-effectiveness and be reassured that they are considering information that poses no risk of discrimination against any patient group. If ICER's analysis finds a major difference in these two measures, we will include specific language in our report describing the underlying characteristics of the treatment and the condition that lead to the difference.

The QALY remains the gold standard in cost-effectiveness analyses for many reasons, and a systematic departure from using the QALY would risk undervaluing treatments that improve the quality of life more than other alternatives for that condition. By drawing greater attention to the analysis of a treatment's evLYG, however, ICER hopes to provide peace of mind to concerned patients and policymakers, while furthering the ability of cost-effectiveness analysis to support explicit, transparent discussions in the U.S. on how best to align a drug's price with its benefits for patients.

life **exactly the same** across all diseases, regardless of the patient population's age, severity of illness, or level of disability.

WITH evLYG, ONE ADDED YEAR = ONE ADDED YEAR



ICER welcomes broad stakeholder input on the QALY and the evLYG as we update our value assessment framework in 2019.

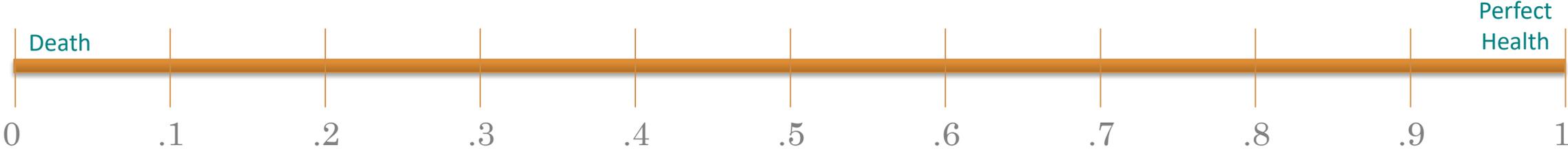
Why the evLYG Doesn't Fix the Problem

- The evLYG **partially** mitigates the life-extension problem – if insurers use it.
- But it still offers payers a means of refusing access to an effective and beneficial drug
- The evLYG doesn't address the undervaluing of quality of life improvements or ignoring clinical knowledge.
- QALY-based systems are less effective than condition-specific means of assessment



Disability weights create arbitrary standards for measuring quality of life improvements

What's the value of your life?



Severe MS



Moderate MS



Mild MS

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Expanded Disability Status Scale (EDSS)

Score	Description
1.0	No disability, minimal signs in one FS
1.5	No disability, minimal signs in more than one FS
2.0	Minimal disability in one FS
2.5	Mild disability in one FS or minimal disability in two FS
3.0	Moderate disability in one FS, or mild disability in three or four FS. No impairment to walking
3.5	Moderate disability in one FS and more than minimal disability in several others. No impairment to walking
4.0	Significant disability but self-sufficient and up and about some 12 hours a day. Able to walk without aid or rest for 500m
4.5	Significant disability but up and about much of the day, able to work a full day, may otherwise have some limitation of full activity or require minimal assistance. Able to walk without aid or rest for 300m
5.0	Disability severe enough to impair full daily activities and ability to work a full day without special provisions. Able to walk without aid or rest for 200m
5.5	Disability severe enough to preclude full daily activities. Able to walk without aid or rest for 100m
6.0	Requires a walking aid – cane, crutch, etc. – to walk about 100m with or without resting
6.5	Requires two walking aids – pair of canes, crutches, etc. – to walk about 20m without resting
7.0	Unable to walk beyond approximately 5m even with aid. Essentially restricted to wheelchair; though wheels self in standard wheelchair and transfers alone. Up and about in wheelchair some 12 hours a day

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6.0	Requires a walking aid – cane, crutch, etc. – to walk about 100m with or without resting
6.5	Requires two walking aids – pair of canes, crutches, etc. – to walk about 20m without resting
7.0	Unable to walk beyond approximately 5m even with aid. Essentially restricted to wheelchair; though wheels self in standard wheelchair and transfers alone. Up and about in wheelchair some 12 hours a day
7.5	Unable to take more than a few steps. Restricted to wheelchair and may need aid in transferring. Can wheel self but cannot carry on in standard wheelchair for a full day and may require a motorised wheelchair
8.0	Essentially restricted to bed or chair or pushed in wheelchair. May be out of bed itself much of the day. Retains many self-care functions. Generally has effective use of arms
8.5	Essentially restricted to bed much of day. Has some effective use of arms retains some self-care functions
9.0	Confined to bed. Can still communicate and eat
9.5	Confined to bed and totally dependent. Unable to communicate effectively or eat/swallow
10.0	Death due to MS

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HAMILTON DEPRESSION RATING SCALE (HAM-D)

(To be administered by a health care professional)

Patient Name _____

Today's Date _____

The HAM-D is designed to rate the severity of depression in patients. Although it contains 21 areas, calculate the patient's score on the first 17 answers.

1. **DEPRESSED MOOD**
(Gloomy attitude, pessimism about the future, feeling of sadness, tendency to weep)
0 = Absent
1 = Sadness, etc.
2 = Occasional weeping
3 = Frequent weeping
4 = Extreme symptoms

2. **FEELINGS OF GUILT**
0 = Absent
1 = Self-reproach, feels he/she has let people down
2 = Ideas of guilt
3 = Present illness is a punishment; delusions of guilt
4 = Hallucinations of guilt

3. **SUICIDE**
0 = Absent
1 = Feels life is not worth living
2 = Wishes he/she were dead
3 = Suicidal ideas or gestures
4 = Attempts at suicide

4. **INSOMNIA - Initial**
(Difficulty in falling asleep)
0 = Absent

6. **INSOMNIA - Delayed**
(Waking in early hours of the morning and unable to fall asleep again)
0 = Absent
1 = Occasional
2 = Frequent

7. **WORK AND INTERESTS**
0 = No difficulty
1 = Feelings of incapacity, listlessness, indecision and vacillation
2 = Loss of interest in hobbies, decreased social activities
3 = Productivity decreased
4 = Unable to work. Stopped working because of present illness only. (Absence from work after treatment or recovery may rate a lower score).

8. **RETARDATION**
(Slowness of thought, speech, and activity; apathy; stupor.)
0 = Absent
1 = Slight retardation at interview
2 = Obvious retardation at interview
3 = Interview difficult
4 = Complete stupor

Hamilton Depression Rating Scale

TOTAL ITEMS 1 TO 17: _____

0 - 7 = Normal

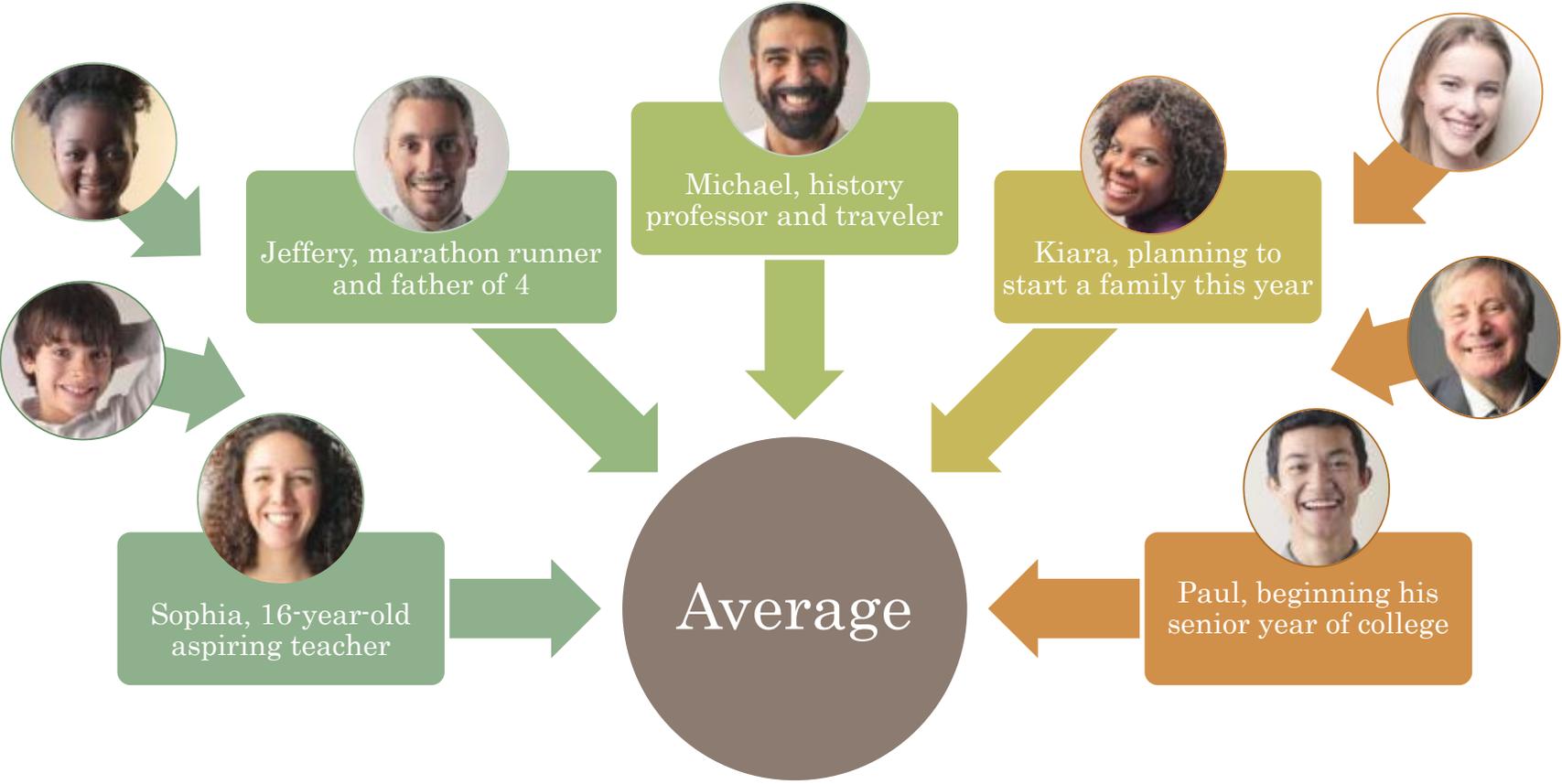
8 - 13 = Mild Depression

14-18 = Moderate Depression

19 - 22 = Severe Depression

≥ 23 = Very Severe Depression

QALYs ignore differences in patient needs and preferences because they are based on averages



Different People Respond Differently to the Same Drugs

**For many conditions, such disparities
are reflected in clinical knowledge –
but not yet in research literature**

Limitations of QALY-Based Cost Effectiveness Analysis



QALYs are acknowledged by experts to discriminate against people with disabilities by placing a lower value on their lives



QALYs devalue the full range of benefits and costs that matter to patients through simplified “disability weight” systems



QALYs ignore important variability in patients’ individual needs and preferences because they are developed using population averages

What is the Institute for Clinical Economic Review?

ICER Value Assessments

- Conducts cost effectiveness studies for insurers using the cost-per-QALY methodology, with a new emphasis on first-in-class therapies.
- Past studies in rare disease include:
 - Spinal Muscular Atrophy
 - Cystic Fibrosis
 - Tardive Dyskenesia
 - Voretigene Neparvovec (retinal disease)
- Potential studies in rare disease in 2020
 - Beta thalassemia (blood disorder)
 - Cystic Fibrosis
 - Hemophilia A
 - Multiple Sclerosis
 - Sickle Cell
 - Wet age-related macular degeneration

ICER's Evolution



ICER Definition of Rare and Ultra-Rare

- In 2017, ICER created a new “ultra-rare” category of diseases with patient populations of less than 10,000, a classification which does not correspond to any accepted definition of rare or ultra-rare diseases. It drew scrutiny from the patient advocacy community, including the National Organization for Rare Disorders (NORD).
 - “NORD is very concerned with ICER’s proposed division of rare diseases into ultra-rare and non-ultra- rare conditions, and opposes this proposal. For decades, NORD has opposed efforts to create an ultra- rare category in various settings. For example, NORD has opposed creating an ultra-orphan category within FDA regulatory review of orphan therapies, public and private reimbursement policy for orphan therapies, and incentives for orphan drug development. Invariably we have asserted that creating an ultra-orphan subcategory will do more harm to the rare diseases that do not fall within that category than good for the rare diseases that do.”

Impact on Innovation

- ICER uses a short-term affordability threshold in its framework. This sets a threshold for spending on all new medicines, regardless of the patient populations or burden of the condition. This has far reaching consequences for future patients and innovation.
 - This creates an inverse relationship between the number of FDA approvals per year and the budget impact, if this arbitrary budget cap were implemented, it could have the effect of limiting companies from continuing to invest in novel treatments
 - This only takes into consideration the price of the drug, not the health systems savings of new, potentially curative treatments

Reliance on Discriminatory Methods

- Cost-effectiveness analyses rely on the use of discriminatory QALYs and similar one-size-fits-all summary metrics.
 - Amed Alliance recommends against relying on quality-adjusted life year (“QALY”) measures to evaluate orphan treatments. The use of QALY measures to evaluate ultra-rare diseases raises significant ethical concerns. For example, individuals with rare diseases should have the same access to treatment as individuals with common diseases and conditions, regardless of whether the QALY gain is large. Yet, QALY measures put a price tag on the value of a human life that merely reflects the individual’s diagnosis and deems those with chronic, debilitating, and rare conditions, as being worth less than those with common diseases. They treat individuals’ lives and health as a commodity and ignore patients’ and practitioners’ individualized concept of the value of treatment.

Premature Assessments

- ICER's rush to deliver payers and policymakers value assessments immediately upon FDA approval has led to hasty reviews based on early assumptions, oversimplified models, and incomplete data. By prioritizing speed over quality, especially for first-in-class treatments, ICER gives payers and policymakers flawed information to justify non-coverage – even where no other treatment choices exist.
 - Plasma Protein Therapeutics Association: Constructing a cost-effectiveness model may be very challenging if based on the results of a clinical trial in ultra-rare conditions, because of insufficient knowledge of how the effect on the surrogate endpoints will translate into the effect on clinically relevant endpoints such as morbidity and mortality, in a long term. This uncertainty, multiplied by frequently seen evidentiary uncertainty shown within the clinical trial, may significantly limit the practical value of long-term cost-effectiveness modeling.

Failure to Meaningfully Engage Patients or Consider Outcomes that Matter

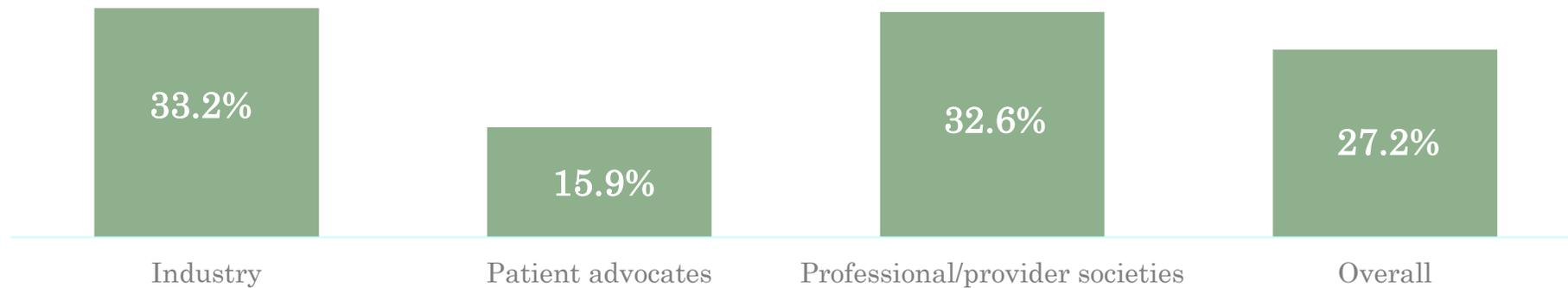
- ICER chooses to leave patients, caregivers and clinicians who have firsthand experience with the condition under review out of the deliberation and voting process. Their failure to include experts who can provide relevant and meaningful insight about the impact of specific conditions demonstrates how little ICER respects the value of patient and clinician voices.
 - CureSMA: This analysis is further weakened by its lack of patient perspective...vastly different from current approaches to the drug approval processes and safety protocols at the FDA, research priorities and protocols at the NIH, and the philosophy reflected in recent milestone legislation, the 21st Century Cures Act...ICER assigns benefit to the patient only if the drug allows for obtaining milestones such as sitting or walking. Meanwhile patients have reported...the great value in abilities that allow for more independence and activities of daily living... It should also be noted that even incremental increases in a patient's motor abilities may alleviate the stresses and challenges involved in caregiving by allowing patients greater ability for self-care.

Lack of *Meaningful* Patient Engagement in Development of ICER Studies

Despite ICER acknowledging a majority of comments, only 27 percent were incorporated into final reports.

Comments from patient advocates were half as likely to be incorporated compared to other stakeholder groups.

Percentage of Stakeholder Comments Incorporated Into ICER Final Evidence Reports



All comments: Industry, N=208; patient advocates, N=157; professional/provider societies, N=95

QALYs Have Historically Been Rejected by Policymakers

- The ACA explicitly prohibits PCORI from using the cost-per-QALY to determine effectiveness, and further restricts use in Medicare to determine coverage, reimbursement, or incentive programs.
- In 1992, HHS rejected Oregon's prioritized list of covered services for Medicaid citing the potential for violating the ADA due to use of QALYs and cost effectiveness.

Oregon Health Plan

“Oregon's plan in substantial part values the life of a person with a disability less than the life of a person without a disability. This premise is discriminatory and inconsistent with the Americans with Disabilities Act.

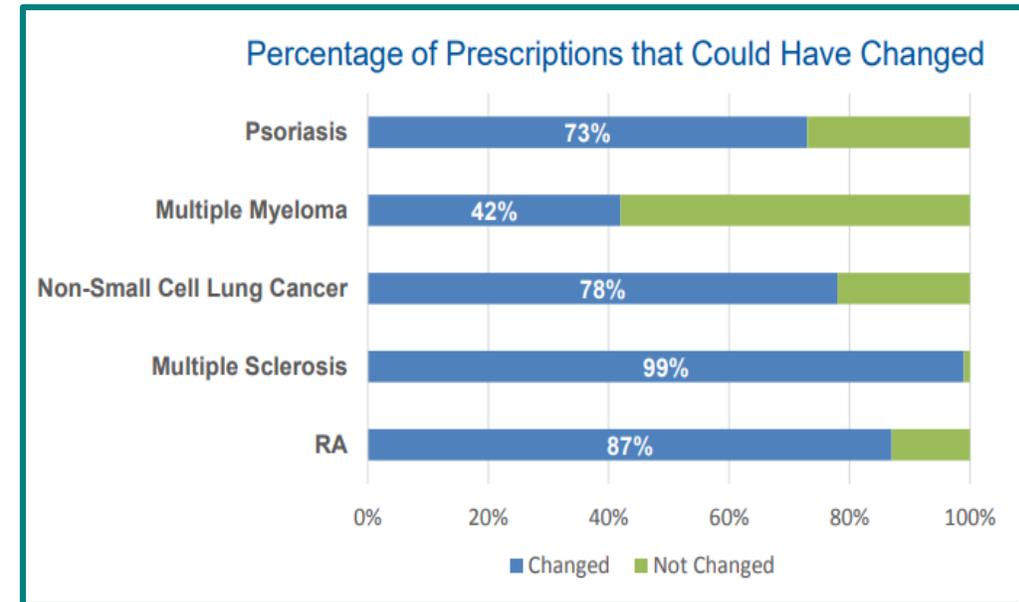
Given the outpouring of comments received by this department and the White House on this issue, I am confident in saying Oregon would have been sued if we had approved the waiver, preventing Oregon from implementing the plan for years. Accordingly, we requested revision of the proposal to remove factors impermissible under the Americans with Disabilities Act.”

– Louis Sullivan, HHS Secretary, Letter in the New York Times, Aug 13, 1992

Why do QALYs Matter? Medicaid Access to Care!

A significant number of patients in five disease areas would lose access to treatments they are currently on, which their doctors deemed best for them, if Medicaid began utilizing an ICER-based formulary.

- Between 42% and 99% of patients across five disease areas would be required to switch treatments if Medicaid used ICER's judgement to determine patient access.
- Essentially all Medicaid patients with MS would be forced to switch treatments, since ICER has deemed only one medication "high value" for MS, and it accounts for only .04% of prescriptions.
- 87% of Rheumatoid Arthritis prescriptions would change if Medicaid used an ICER-based formulary.

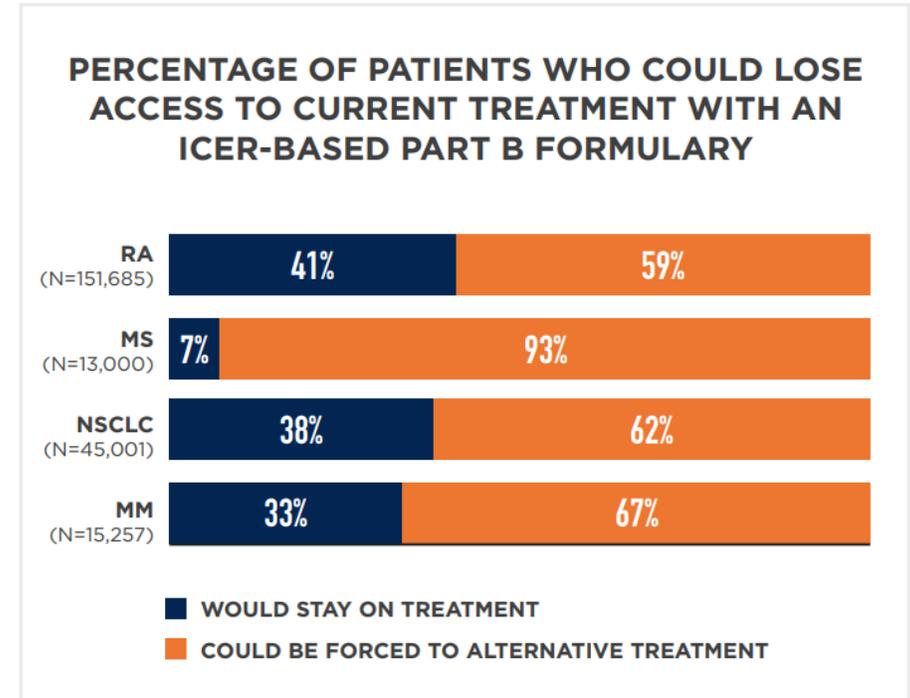


Why do QALYs Matter?

Medicare Part B Access to Care!

More than half of Medicare Part B beneficiaries in the selected disease areas would lose access to needed care if ICER's judgments were used as a government value standard.

- Between 55% and 62% of patients across four disease areas would be required to switch treatments if Medicare used ICER's judgement to determine patient access in Medicare Part B.
- The switch would most impact MS patients most significantly – nearly 93% of patients would lose access to the treatment their physician prescribed.



Implications for Veterans and Military

- In 2017, ICER announced a partnership with the VA “to integrate ICER reports into the VA formulary management process of evaluating the comparative clinical effectiveness and value of drugs.”
 - Over 40 organizations signed a letter expressing concern to the VA
- In a recent review, PIPC found that ICER evaluated 54 drugs at low-intermediate value and 42 of them are not covered on the national VA formulary
- DOD is now implementing Section 702 of the FY 2018 NDAA which seeks to “pay for value” by allowing drugs to be excluded from the formulary that “provides very little or no clinical effectiveness to covered beneficiaries and the Department under the program.”
 - It is not clear who defines “clinical effectiveness” and “value” for DOD

Federal Policy Threats

- The Administration proposed an International Price Index
- The House of Representatives is considering a “binding arbitration” model that would reference ICER studies
- States are considering reference to ICER studies under Medicaid
- The VA has partnered with ICER in determining its formulary
- DOD/Tricare considering “value-based” coverage restrictions

IPI: Experience in Other Countries

Worse Outcomes

For breast, colon, lung and prostate cancers, 5-year survival rates are higher in U.S. than those in Canada, France, Germany, Italy, Japan and the U.K.

Fewer Options

Almost 80% of cancer medicines reviewed by U.K. health officials between 2007 and 2014 had some form of access restriction.

Slower Access

U.S. patients have access to cancer medicines on average 2 years earlier than patients in other developed countries

News ▶ Health ▶ NHS

Miracle drug that could prolong this four-year-old's life 'too costly' for NHS

The one medication that could slow down Francesca's degenerative condition has been rejected as 'not cost effective' by drugs regulator

Cystic fibrosis sufferers denied life-prolonging drug by NHS

The maker of a costly treatment now licensed for sufferers as young as six is trying to strike a deal with the health service

See www.pipcpatients.org/access to learn more about other countries.



Developments in States

- The President’s budget proposed a proposed 5-state demonstration inviting states to “make drug coverage decisions that meet state needs.”
- CMS opened door to restricted coverage in their response to MA proposed waiver:
 - “Adopting a closed formulary with at least a single drug per therapeutic class would enable MassHealth to negotiate more favorable rebate agreements with manufacturers... the majority of commercial pharmacy benefit managers (PBMs) have adopted such closed formularies, which allow them to customize their drug offerings based on clinical efficacy and cost considerations.”
- New York: Passed legislation allowing for use of “value” assessments to determine supplemental rebates, also allowing for drugs with multiple in a class to be excluded from formularies. This year’s budget gave more explicit authority to use a third party like ICER to determine that “target” price.
 - Letter signed by over 40 groups opposing provision in budget
- Massachusetts: Proposed policies modeled on New York reference “value”
 - Disability groups actively opposing and calling for a ban on use of QALYs
- California: The Legislative Analyst Office has provided recommendations to consider the New York model.
- Other states are also considering similar policies referencing third party value assessments to determine reimbursement and coverage

Principles for Value Assessment

- Acknowledge diversity and differences among patients and people with disabilities
- Should not be misused by payers and policymakers to limit patient access
- Developed using transparent processes and methods
- Meaningfully engage with patient and provider organizations
- Rely on a range of sound, patient-centered sources of evidence
- Address costs and benefits that matter to the patient
- Produce evidence on the value of treatments based on patient-centered outcomes

Advocate!

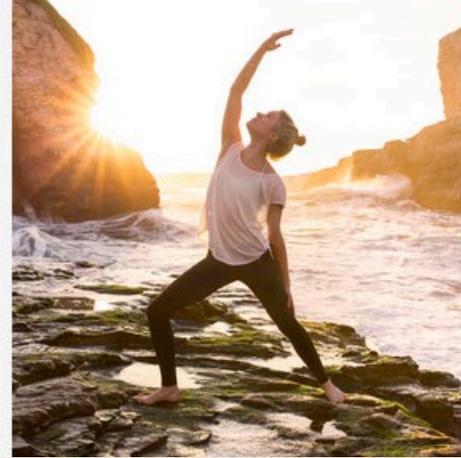
- Add your logo to Value Our Health!
- Educate fellow advocates on these complex issues that provide a foundation for discrimination and denied coverage for those not fitting the average.
- Question payers basing coverage on “value” – ask how they define value.
- Tell state policymakers: We oppose use of cost effectiveness thresholds as the basis for Medicaid coverage policies.
- Tell federal policymakers: We oppose policies undermining federal law protecting patients and people with disabilities from discriminatory metrics in Medicare.
- Tell employers, insurers and PBMs: We oppose use of discriminatory cost effectiveness.

To stay tuned, get PIPC updates! www.pipcpatients.org



Value Our Health

www.valueourhealth.org



CFRI: Your Partner in Living

Mission Statement: CFRI exists to fund research, to provide educational and personal support, and to spread awareness of cystic fibrosis, a life-threatening genetic disease.

CF Stats: 30,000 people affected in the United States; systemic disease most known for causing progressive & fatal lung disease; median age of death, 30 years.

ICER is self-ordained. Their credibility comes from those who will benefit from their analysis.

ICER's claims of transparency and inclusion are a myth.

ICER does not understand the challenges faced by the rare disease community.

ICER's flawed methodology has no place when reviewing rare disease therapies.



Rare Disease Statistics:

- There are approximately 7,000 types of rare disorders, with more being discovered each day;
- 30 million people in the U.S. are living with rare diseases (1 out of 10 Americans);
- In the United States, a condition is considered “rare” if it affects fewer than 200,000 persons;
- 80% of rare diseases are genetic in origin;
- Approximately 50% of those affected by rare diseases are children, and 30% of these children will not live to see their 5th birthday;
- Rare diseases are responsible for 35% of deaths in the first year of life;
- While there are 500 FDA-approved therapies for different rare diseases, and nearly 600 medications currently in development, 95% of rare diseases have not one single FDA-approved drug treatment.

CFRI's Experience: ICER Review of CFTR Modulators

- **November 2017: Public comments on the draft scoping document related to the Effectiveness and Value of Modulator Treatments for Cystic Fibrosis**
- **April 2018: Comments on Draft Evidence Report**
- **May 2018: Public Comment at the Midwest Comparative Effectiveness Public Advisory Council in St. Louis MO.**
- **May 2018: Post-Meeting Press**
- **The Future: Utilize lessons learned for the next round.**

ADVICE TO CONSIDER:

“Pay no attention to that man behind the curtain!”

- The Wizard of Oz

- **Do not be intimidated by ICER;**
- **Make sure your organization is ready for policy positions and actions;**
- **Assign a point person within your organization;**
- **Educate your elected officials about the unique challenges faced by the rare disease community, and why ICER’s analyses may have fatal implications;**
- **Point out flawed methodology clearly and repeatedly.**

MORE ADVICE...

- **Make sure you are informed when/if ICER decides to focus on your rare disease drug;**
- **Do not ignore calls for input, but do not be used to provide credibility;**
- **You do not need to be an expert on drug pricing;**
- **Rally your community using email, phone calls, social media posts, etc; flood ICER with letters;**
- **Bring in the media: Send press releases to digital and print media outlets, post links on social media, and involve your fellow disease group peers;**
- **Keep informed of ICER's influence on public/private payers and be prepared to respond.**



Research for Living ~ Partners for Life

Thank you!

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www.valueourhealth.org

