The Perils of QALYs: Addressing Disability Discrimination

September 10, 2019
Overview

• What is a Quality-Adjusted Life Year (QALY)?
• What is the Institute for Clinical Economic Review?
• What is the History of Use of QALYs?
• How are ICER’s QALY-Based Studies Used in Coverage?
• What are Emerging Threats to Expand Use of QALYs?
• What is the Response?
What is a QALY Exactly?

September 10, 2019

New York Association on Independent Living Statewide Conference and Expo
What is a QALY?

• A Short History of Value-Based Purchasing: “how do we allocate finite healthcare resources to best improve population health”
• QALYs “value” the impact of “treatment” on years of life and assign a numerical value:
  • 1 QALY = 1 year in ’perfect health’
  • 0 QALY = Death
  • Disabled or sick lives fall somewhere between 0 and 1
What is a QALY?

An Example: Bipolar Disorder

Manic State: 0.492 (0.341 - 0.646)
Residual State: 0.032 (0.018 - 0.051)
Depressive State: 0.396 (0.267 - 0.531)

Anxiety disorders, currently without symptoms:
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What is a QALY?

What’s The Value of Your Life?

Person with Cancer  Person with Rheumatoid Arthritis  Person with Diabetes
Challenges with QALYs

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.”

In other words, isn’t this just discrimination?
Challenges with QALYs

- **Inaccuracy**: research suggests that the non-disabled population systematically overestimates the burden of life with disability
- **Undervaluing of quality of life**: even models where people with disabilities self-report may find it very hard to demonstrate sufficient treatment efficacy gains
Challenges with QALYs

• **Ineffective**: 
  • QALY-based systems are less effective than condition-specific means of assessment, like:  
    • Expanded Disability Status Scale (EDSS)  
    • Various mental health scales  
  • QALYs ignore differences in patient needs and preferences because they are based on averages
Challenges with QALYs

• Recently, ICER introduced the Equal Value of Life Years Gained (evLYG)
  • evLYG does not eliminate use of QALYs
  • It is a new number that can be considered in conjunction with QALYs
  • The evLYG doesn’t address the inaccuracies undervaluing of quality of life improvements
  • Payers can still deny on the basis of QALYs
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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 include:
  - Spinal Muscular Atrophy
  - Cystic Fibrosis
  - Tardive Dyskenesia
  - Voretigene Neparvovec (retinal disease)

- Potential studies in 2020:
  - Beta thalassemia (blood disorder)
  - Cystic Fibrosis
  - Hemophilia A
  - Multiple Sclerosis
  - Sickle Cell
  - Wet age-related macular degeneration
ICER’s Evolution

- ICER Founded
- ICER Reference in Medicare Part B Payment Demonstration
- ICER / Department of Veterans Affairs Collaboration
- ICER Receives $13.9M Grant from the Arnold Foundation
- ICER Collaboration with New York Drug Utilization Review Board
- CVS/Caremark announces reliance on ICER reports

- 2006
- Mar 2016
- Jun 2017
- Oct 2017
- March 2018
- May 2018
Flaws in ICER’s Methods

• Reliance on Discriminatory Methods
  – Use QALYs and similar one-size-fits-all summary metrics.
  – Place a lower value on people with disabilities and serious chronic conditions
  – Sidesteps ethical problems related to using QALYs in health care decision-making.

• Failure to Meaningfully Engage Expert Stakeholders
  – Leaves patients, caregivers and clinicians who have firsthand experience with the condition under review out of the deliberation and voting process.

• Failure to Consider Outcomes that Matter to Patients and People with Disabilities
  – Values a treatment strictly from the health system and insurer perspectives. This can lead to situations where it is more “valuable” not to provide care for some patients because to do so would not be “cost-effective.”

• Premature Assessments
  – 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.

• Lack of Transparency to Patients and People with Disabilities
  – Assessment process is a black box, leaving patients and people with disabilities in the dark on the assumptions used and important limitations that may have impacted the results.
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

- **Industry**: 33.2%
- **Patient advocates**: 15.9%
- **Professional/provider societies**: 32.6%
- **Overall**: 27.2%

*All comments: Industry, N=208; patient advocates, N=157; professional/provider societies, N=95*
Different People Respond Differently to the Same Drugs
For many conditions, such disparities are reflected in clinical knowledge – but not yet in research literature.
What is the History of Use of QALYs?
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'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.”

The President’s budget proposed a 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.”

Other state Drug Utilization Review Boards referencing ICER:
- Oklahoma Example: ICER’s QALY-based studies were used as part of deliberations to impose prior authorization requirements on Takhzyro, medication for Hereditary Angioedema, and Zolgensma, medication for spinal muscular atrophy.
New York State Medicaid Drug Cap

• Drug cap process:
  – If projected spending exceeds the "drug cap" then the state can look for additional rebates
  – The Dept of Health identifies drugs for review by the Drug Utilization Review Board (DURB)
    • At this time the DURB has only 1 of 3 consumer seats filled, themselves a small minority of the 23-seat board
  – The DURB has used ICER as the benchmark for value in the past
    • This year's budget gave more explicit authority to use a third party like ICER to determine that “target” price
developed letter signed by over 40 groups opposing provision in budget
  – For a manufacturer that did not agree to target supplemental rebates, the commissioner can
    impose utilization management on all of the manufacturer’s drugs, not just the one targeted for
    supplemental rebates.
    • Utilization management tools include requiring prior authorization, directing managed care plans to stop covering
the drugs, and promoting “cost effective” and clinically appropriate drugs in place of them, such as indicated by
ICER.
Private Plans Reference QALYs for Utilization Management

- 59% of payers indicated that they used ICER reports (likely moreso today as ICER is conducting more studies)
  - May 2016 Survey conducted by Dymaxium
- Payers report using ICER reports to guide their drug information process and frequently using ICER reports to inform prior authorization and step therapy requirements.
  - One payer specifically commented that ICER reports are reviewed in nearly every relevant clinical and value subcommittee.
  - Study by ICON
- PCSK9 Example: ICER reviewed PCSK9 Inhibitors and called them “low value” generally and “intermediate value” for certain patients. In 2017, payers rejected claims for PCKSK9 inhibitors for 63 percent of patients with presumed FH and 58 percent of patients with established atherosclerotic cardiovascular disease (ASCVD) despite sub-optimal low-density lipoprotein cholesterol (LDL-C) levels on statins. Conversely, drugs not reviewed by ICER to treat the same conditions, were rejected 9 percent and 8 percent of the time, respectively.
  - Data from the Familial Hypercholesterolemia (FH) Foundation
What are Implications of Using QALY-Based Studies in Coverage?
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

• 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
**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.

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See [www.pipcpatients.org/access](http://www.pipcpatients.org/access) to learn more about other countries.
What are Emerging Threats?
Looming Federal Policy Threats

- The Administration proposing 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
- 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
Incentives for State-Based Activities

• The President’s budget proposed a 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

• 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
Massachusetts Case Study
State Context

• Overwhelmingly Democratic legislature, Republican governor (Baker) who is seen as centrist and pro-business.
• MassHealth—state Medicaid plan. Seen as healthcare laboratory, prides itself on innovation.
  – Early adopter of Duals demonstration under ACA; One Care plan developed with significant input from disability community.
  – Recent, somewhat controversial shift for general Medicaid to ACOs.
• Large and energized healthcare advocacy community
• Growing concern over prescription spending—14% growth
In 2017, Baker administration proposes giving the state authority to exclude drugs from the MassHealth formulary as a negotiating tactic.

Would have been based on prices set based on value analysis, likely QALYs.

Significant concerns raised by advocates regarding access

Rejected in 2018 by CMS because it would have required pulling out of the rebate program; state was invited to resubmit on that basis. Would have meant giving up all rebates and negotiating everything down from list price, but certain drugs could have been excluded.
2018-19: Multiple Proposals

• At the 2018 Health Policy Commission (HPC) Hearing, multiple proposals advanced by various parties
  – Return to 2017 formulary closure
  – ICER suggests “value-based formulary”
  – Proposal based on New York model
  – Transparency and disclosure measures

• No disability voices represented, only one consumer organization—Health Care for All
DPC’s Approach: Leverage Relationships and Moral Force

- In partnership with PIPC’s Ari Ne’eman, DPC engaged both state policymakers and Health Care for All in ongoing talks.
- We leveraged strong existing relationships and made it clear that the disability community needed to be represented.
- Conducted and shared in-depth research and recruited experts.
Position the Disability Community as Lynchpin

• States and advocates want to take on drug prices—for understandable reasons!

• Our goal isn’t to stop reform, it’s to steer it.

• Position disability organizations as valuable voices. We can either rebut or reinforce the idea that a proposal will hurt patients
• Governor’s Proposal included no access restrictions
  – Primary focus is on transparency, accountability, “public shaming” of drug companies.
  – They’ve made public commitment to not use metrics that discriminate against people with disabilities
  – They’ve asked for our support. In return, we’ve asked for an explicit rejection of QALYs and a disability seat on the state’s Health Policy Commission
Results contd.

• Final budget included key transparency language
  – We successfully advocated for amendment language that increases scrutiny on third-party metrics
    • (g) If the commission relies upon a third party to provide cost-effectiveness analysis or research related to the proposed value, such analysis or research shall also provide, but not be limited in scope to, (i) a description of the methodologies and models used in its analysis; (ii) any assumptions and potential limitations of research findings in the context of the results; and (iii) outcomes for affected subpopulations that utilize the drug.

• Multiple standalone bills introduced that would address QALY issue
Results contd.

• Health Care for All Proposal is disability friendly
  – Relied on different analytical approach that does not use ICER
  – Ensures consumers will have access to drugs if negotiations break down
  – Includes language banning metrics that devalue the lives of people with disabilities
  – In return, DPC signed on as a coalition member
Today, the Massachusetts Joint Committee on Public Health is considering 2 bills related to value assessment:

- Language calling for value assessments to be transparent, free of conflicts of interest and to include the voices of patients and other experts.
- Advocates will testify in support of adding an explicit ban on use of QALYs.

Prohibition on Reliance on Discriminatory Measures. The executive office shall not develop or employ a dollars-per-quality adjusted life year (or similar measure that discounts the value of a life because of an individual’s disability) as a threshold to establish what type of health care is cost effective or recommended. The executive office shall not utilize such an adjusted life year (or such a similar measure) as a threshold to determine coverage, reimbursement, or incentive programs.
The Disability Response
Consortium of People with Disabilities

• CCD Developed principles for drug pricing:
  – Oppose the use of Quality Adjusted Life Years
  – Support Limits on Cost Sharing
  – Oppose Discriminatory Benefit Design
  – Don’t Use Access to Care as Leverage in Negotiations
  – Support and Strengthen Programs that Provide Access for People with Disabilities
  – Support Access to Generics, but not at Expense of Those who Need Brand Name Medications
Support for CCD Principles

Allies for Independence
American Association of People with Disabilities
American Association on Health and Disability
American Association on Intellectual and Developmental Disabilities
American Medical Rehabilitation Providers Association
American Physical Therapy Association
American Therapeutic Recreation Association
Autism Society of America
Autistic Self Advocacy Network
Brain Injury Association of America
Center for Public Representation
Disability Rights Education & Defense Fund

Easterseals
Epilepsy Foundation
Justice in Aging
Lutheran Services in America-Disability Network
National Alliance on Mental Illness
National Association of Councils on Developmental Disabilities
National Council for Behavioral Health
National Down Syndrome Congress
National Health Law Program
The Arc of the United States
United Spinal Association
Value Our Health: 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