



March 7, 2023

Additional Questions for the Record

“Lives Worth Living: Addressing the Fentanyl Crisis, Protecting Critical Lifelines, and Combatting Discrimination Against Those with Disabilities.”

Kandi Pickard
President and CEO
National Down Syndrome Society



The Honorable Gus Bilirakis

1. Mrs. Pickard, the QALY methodology seems rather subjective: Could you please elaborate on the arbitrary nature of QALYs?

Quality Adjusted Life Years (QALYs) discriminate against people with disabilities in several ways. QALYs use numeric indicators called “utilities” to assign a value to life lived in a particular health state. These utilities are created through broad population surveys in which participants, many of whom do not understand the lived experiences of people with disabilities, must assign a value to health states in which they may have limited knowledge of and likely have not directly experienced. Participants may perceive individuals with disabilities to have a lower quality of life based solely on their diagnosis and thus, assign them a lower utility. This framework is inherently flawed as it allows for the biases of survey participants to undermine the assessment. As a result of QALYs, people with disabilities are often seen as too expensive or not worth treating. As a parent of a child with Down syndrome, I know firsthand that my son’s diagnosis does not mean his life is any less valuable than my children without disabilities.

2. What are the ethical implications of the Institute of Clinical and Economic Review (ICER) model and its utilization of QALYs?

The Institute for Clinical and Economic Review (ICER) conducts QALY-based cost-effectiveness assessments, which payers acknowledge relying on for formulary decisions.¹ These assessments have led to restricted and delayed access to treatments. In 2014, ICER evaluated Hepatitis C treatments and found them to be low value to the health care system despite providing high impact to patients. Following this evaluation, patients struggled to receive coverage for these treatments.² In 2015, ICER conducted an assessment of PCSK9s, a treatment for heart disease, and found them not to be cost-effective which resulted in denial of coverage at a high rate.³ Both of these treatments yielded profound benefits for patients yet, based on the flawed and discriminatory assessments provided by ICER, were ultimately restricted. Parents of children with Duchenne Muscular Dystrophy have also reported having ICER’s assessment cited to them as a rationale for denying coverage of eteplirsen.⁴

Most recently, CMS relied upon a report from ICER that used QALYs and similar one-size-fits all metrics in its national coverage determination for Aduhelm, the first treatment approved for Alzheimer’s disease. The initial coverage determination excluded individuals with disabilities. This was particularly concerning as individuals with Down syndrome have a heightened lifetime risk – higher than 90 percent – of developing Alzheimer’s disease.⁵ Access to treatments for this

¹ <https://www.xcenda.com/insights/ispur-2022-poster-impact-icer-assessments-payer-decision-making>

² http://icerorg.wpengine.com/wp-content/uploads/2020/10/CTAF_HCV2_Final_Report_013015.pdf

³ <https://familyheart.org/research-circ-ce-data>

⁴ <https://www.bostonglobe.com/2021/11/22/opinion/how-health-care-systems-do-not-support-patients/>

⁵ McCarron, M., McCallion, P., Reilly, E., Dunne, P., Carroll, R., & Mulryan, N. (2017). A prospective 20-year longitudinal follow-up of dementia in persons with Down syndrome. *Journal of intellectual disability research* : JIDR, 61(9), 843–852. <https://doi.org/10.1111/jir.12390>



debilitating disease is paramount to our community, and we will continue to work with Members of Congress and this Committee to ensure individuals with disabilities are not left out of the conversation.

3. What lessons should the United States learn from the controversial utilization of QALYs in other global health care systems?

Many countries, including our friends in the UK and Canada heavily rely on QALYs to determine who is worth treating and who is “too expensive” thus determining which medicines or treatments are available to patients. For example, from 2016 to 2019, the UK used QALYs to restrict access to the first ever approved treatment for cystic fibrosis, and there are still severe limitations put on the use of disease modifying drugs for Cystic Fibrosis in countries that rely on QALY-based HTA, including Canada and New Zealand. A 2018 Avalere Health study found that of over 329 health technology assessments of cancer drugs between 2013 and 2017, the National Institute for Health and Care Excellence (NICE) in the U.K recommended access restrictions for nearly 70% of the drugs it assessed and rejected 22%.⁶ Unfortunately, as indicated above, these metrics are used here in the United States as well for federal health program coverage determinations.

The Honorable Michael Burgess

1. How else, besides using QALY measurements, can Congress determine cost-effectiveness for care while also acknowledging the inherent value of every person?

According to a 2019 report issued by the National Council on Disability, no single alternative exists that serves all the functions of the QALY as a sole metric for determining value.⁷ The NCD and entities such as the Disability Rights Education and Defense Fund (DREDF) have recommended that alternative metrics be used jointly, in combination, to understand value from different perspectives, similar to methods like multi-criteria decision analysis (MCDA).⁸ I believe more research is needed to further develop and test alternative methods and frameworks for determining the value of health care treatments. We must also ensure that individuals with disabilities are included in decisions regarding the value of their health care. This will better inform policy makers on the nuances missing from the available evidence base and ensure that the voices of the disability community are kept at the center of this issue.

2. How have QALYs been used previously to restrict access to treatments?

QALYs are routinely used in cost-effectiveness assessments by the Institute for Clinical and Economic Review (ICER), which payers acknowledge relying on for formulary decisions.⁹ This had

⁶ <https://avalere.com/insights/htas-recommendations-for-oncology-have-grown-more-restrictive-over-time>

⁷ https://ncd.gov/sites/default/files/NCD_Quality_Adjusted_Life_Report_508.pdf

⁸ <https://dredf.org/2021/09/23/pharmaceutical-analyses-based-on-the-qaly-violate-disability-nondiscrimination-law/>

⁹ <https://www.xcenda.com/insights/ispor-2022-poster-impact-icer-assessments-payer-decision-making>



led to restrictions on coverage for treatments for conditions such as Hepatitis C and heart disease, even when the treatments yielded significant benefit to the patient.^{10 11}

Many countries, including our friends in the UK and Canada heavily rely on QALYs to determine who is worth treating and who is “too expensive” thus determining which medicines or treatments are available to patients. For example, from 2016 to 2019, the UK used QALYs to restrict access to the first ever approved treatment for cystic fibrosis.

Here in the United States, QALYs continue to be utilized at the state level in many states. The state of Oregon ranks health care services in a prioritized list form more to least important. The Oregon Health Evidence Review Commission (HERC) uses QALYs in its cost-effectiveness formula to determine where treatments fall on the prioritized list.¹² Only services over a certain line are covered, regardless of individual determinations of medical necessity. Oregon is not the only state to rely on this form of health care rationing and states such as Massachusetts, New York, and Minnesota have all sought to enact legislation that relies on value-based assessments in healthcare.

3. What has happened as a result?

As a result of QALYs, access to and coverage of necessary treatments have been routinely restricted for patients who are deemed “too expensive” or “not worth” treating. Most recently, CMS relied upon a report from ICER that used QALYs and similar one-size-fits all metrics in its national coverage determination for Aduhelm, the first treatment approved for Alzheimer’s disease. The initial coverage determination excluded individuals with disabilities. This was particularly concerning as individuals with Down syndrome have a heightened lifetime risk – higher than 90 percent – of developing Alzheimer’s disease.¹³ Access to treatments for this debilitating disease is paramount to our community, and we will continue to work with Members of Congress and this Committee to ensure individuals with disabilities are not left out of the conversation.

All individuals should be valued by our healthcare system and should have access to the treatments that have been prescribed to them by their physicians. My son, and other individuals with disabilities, deserve to access a healthcare system that is free from discrimination and imposed biases.

¹⁰ http://icerorg.wpengine.com/wp-content/uploads/2020/10/CTAF_HCV2_Final_Report_013015.pdf

¹¹ <https://familyheart.org/research-circ-ce-data>

¹² <https://www.oregon.gov/oha/HPA/DSI-HERC/Pages/Prioritization-Methodology.aspx>

¹³ McCarron, M., McCallion, P., Reilly, E., Dunne, P., Carroll, R., & Mulryan, N. (2017). A prospective 20-year longitudinal follow-up of dementia in persons with Down syndrome. *Journal of intellectual disability research* : JIDR, 61(9), 843–852. <https://doi.org/10.1111/jir.12390>