



Quality-Adjusted Life Years and the Devaluation of Life with Disability

Part of the Bioethics and
Disability Series



National Council on Disability

November 6, 2019

National Council on Disability (NCD)
1331 F Street NW, Suite 850
Washington, DC 20004

Quality-Adjusted Life Years and the Devaluation of Life with Disability: Part of the Bioethics and Disability Series

National Council on Disability, November 6, 2019

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National Council on Disability

An independent federal agency making recommendations to the President and Congress to enhance the quality of life for all Americans with disabilities and their families.

Letter of Transmittal

November 6, 2019

The President
The White House
Washington, DC 20500

Dear Mr. President:

On behalf of the National Council on Disability (NCD), I am pleased to submit *Quality-Adjusted Life Years and the Devaluation of Life with Disability*, part of a five-report series on the intersection of disability and bioethics. This report, and the others in the series, focuses on how the historical and continued devaluation of the lives of people with disabilities by the medical community, legislators, researchers, and even health economists, perpetuates unequal access to medical care, including life-saving care.

When health insurance will not cover medically necessary medications and treatments, individuals experience poorer health and a lower life expectancy. Nonetheless, in an effort to lower their healthcare costs, public and private health insurance providers have utilized the Quality Adjusted Life Year (QALY) to determine the cost-effectiveness of medications and treatment. QALYs place a lower value on treatments which extend the lives of people with chronic illnesses and disabilities. In this report, NCD found sufficient evidence of the discriminatory effects of QALYs to warrant concern, including concerns raised by bioethicists, patient rights groups, and disability rights advocates about the limited access to lifesaving medications for chronic illnesses in countries where QALYs are frequently used. In addition, QALY-based programs have been found to violate the Americans with Disabilities Act.

The US government does not have a single comprehensive policy on QALYs. Some federal agencies are banned from utilizing measurement tools like QALYs, while some state and federal partnership programs, such as state Medicaid programs, may. NCD is troubled that health insurance providers, government agencies, and health economists are showing increasing interest in using QALYs to contain healthcare costs despite QALYs' discriminatory effect.

The lives of people with disabilities are equally valuable to those without disabilities, and healthcare decisions based on devaluing the lives of people with disabilities are discriminatory. *Quality-Adjusted Life Years and the Devaluation of Life with Disability* explains QALYs and their effect on the availability of medical care for people with disabilities and chronic illnesses. It makes recommendations to Congress, federal agencies, and public and private insurers directed at rejecting QALYs as a method of measuring cost-effectiveness for medical care and offers alternatives.

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NCD stands ready to assist the Administration, Congress, and federal agencies to ensure that people with disabilities and chronic illnesses have access to the medical care they need.

Respectfully,

A handwritten signature in black ink that reads "Neil Romano". The signature is written in a cursive style with a large initial "N" and a long, sweeping underline.

Neil Romano
Chairman

(The same letter of transmittal was sent to the President Pro Tempore of the U.S. Senate and the Speaker of the U.S. House of Representatives.)

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Acknowledgments

This report is part of a series of reports on bioethics and people with disabilities which was developed through a cooperative agreement with the Disability Rights Education & Defense Fund (DREDF). The National Council on Disability (NCD) appreciates the work of those who contributed their expertise in its development, including Kelly Israel, Policy Analyst, Autistic Self Advocacy Network (ASAN), and Samantha Crane, Director of Legal and Public Policy, ASAN. NCD also appreciates the work of Marilyn Golden, Senior Policy Analyst, DREDF, who shepherded the entire series in cooperation with NCD. We also thank those who participated on the Advisory Panel, in interviews, and in the stakeholder convening, whose knowledge and willingness to share information helped make this series possible.

Executive Summary

Purpose

Healthcare coverage decisions are of vital importance to people with disabilities and their families. If the medications and treatments that extend or improve the lives of people with disabilities are not covered by insurance, they will not have access to needed health care, and will have lower quality of life and lower life expectancy. Public and private insurance providers sometimes attempt to limit their healthcare spending in ways that reduce people with disabilities' access to health care. One of the means by which they do so is by refusing to cover (or by limiting access to) healthcare treatments based on their cost-effectiveness. One metric often used to help calculate cost-effectiveness—quality-adjusted life years (QALYs)—may have a negative impact on the health and welfare of people with disabilities.

QALYs are a number which (theoretically) represents the degree to which a drug or treatment extends life *and* improves quality of life—although quality of life is a difficult concept to define, quantify, and measure. However, QALYs aggregate quality and quantity

of life simply by lowering the value of a year of treatment by the degree to which an illness, disability, or other health condition is perceived to harm the person's quality of life during that year.

There has been increasing interest among national health insurance programs (like Medicaid), private health insurance companies, and pharmacy benefit managers (PBMs; managers of drug

[T]he QALY calculation reduces the value of treatments that do not bring a person back to “perfect health,” in the sense of not having a disability and meeting society’s definitions of “healthy” and “functioning”. . . .

benefits for health insurers) in using QALYs to inform their decisions about which drugs and treatments they will cover. Many individuals, however, have serious concerns with the use of QALYs.

The use of QALYs has been opposed by people with disabilities and disability rights advocates for more than 20 years. Their use is also opposed by some bioethicists and patient rights organizations. These stakeholders fear that use of QALYs undervalues vital treatments that extend or improve the lives of people with disabilities. This is because the QALY calculation reduces the value of treatments that do not bring a person back to “perfect health,” in the sense of not having a disability and meeting society's definitions of “healthy” and “functioning”; uses

simplified assessments of value that do not account for the complexity of patient experience; and does not take into account clinical expertise on rare disorders that may not have an extensive research literature available for use. Other stakeholders—often from the medical, health economics, and health insurance fields—argue that QALYs provide payers with valuable information on a treatment’s potential benefits and costs and aid them in negotiating a reasonable price with the drug (or treatment)’s manufacturers.

Although QALYs have not historically been utilized for benefits and reimbursement decisions in the United States, prominent nonprofit corporations and professional associations are now using QALYs to evaluate the cost-effectiveness of new drugs and treatments. These evaluations now have a strong influence on many private and public health insurers’ decisions about which drugs and treatments they will cover. Additionally, the use of QALYs to inform benefits and coverage decisions in other countries has limited access to lifesaving medications for people with disabilities and those with chronic illnesses.

NCD undertook this report to examine how use of QALYs may impact people with disabilities in the United States and will inform Congress and the executive branch on the ways in which QALYs impact people with chronic illnesses and disabilities’ access to treatment and health care. The report

includes recommendations aimed at ensuring that cost-effectiveness assessments of drugs and medical treatments, considered in benefits and coverage decisions, are fair and nondiscriminatory. NCD’s research team used multiple methods to gather information, including a comprehensive literature review and interviews with experts and stakeholders who understand how QALYs may impact people with disabilities.

Background

Payers in the healthcare context—both private health insurance companies (for example, Anthem) and public health insurers (for example, Medicaid and the Veterans Administration)—typically have a limited amount of money to spend. Payers therefore want to fund treatments or drugs that are of high value and clinical effectiveness. For many payers, a high-value drug or treatment is equivalent to a cost-effective one, but patients may have different opinions on what constitutes value.

A cost-effective treatment is generally considered to be a treatment for which, from the perspective of the payer, the cost of the treatment does not outweigh the health improvements it provides. QALYs are used as one possible measure of the degree to which a treatment improves both quality and quantity of life. A drug or treatment that provides its beneficiaries with more QALYs is considered more effective. Therefore, a drug that provides its

[T]he use of QALYs to inform benefits and coverage decisions in other countries has limited access to lifesaving medications for people with disabilities and those with chronic illnesses.

beneficiaries with more QALYs for less money is considered more cost-effective.

QALYs are used in cost-effectiveness studies, in particular a type of cost-effectiveness study called a cost-utility analysis (CUA), as well as in decision-making tools known as value frameworks. Both are relied on by payers as a source of evidence of a drug or treatment's cost-effectiveness. The final decision made by payers is not dependent on cost-effectiveness as measured in QALYs, but instead is informed by it.

Key Findings

- QALYs have been the subject of considerable ethical debate since they were first invented. The primary ethical issues concern whether or not use of QALYs to calculate the cost-effectiveness of drugs and treatments discriminates against people with disabilities and chronic illnesses, how exactly they do so, and, if they do, whether or not that is ethical. There is not universal agreement on any of these issues. However, NCD has found sufficient evidence of QALYs being discriminatory (or potentially discriminatory) to warrant concern, including: (1) concerns raised by stakeholders in the interviews NCD undertook for this report (including bioethicists, patient rights groups, and disability rights advocates); (2) compelling arguments from prominent bioethicists condemning the use of QALYs; and (3) the inability of patients in countries where

QALYs are used more heavily to obtain coverage of needed health care.

- The Federal Government does not have a single, comprehensive policy on the use of QALYs. The Federal Government has considered increasing its utilization of cost-effectiveness research and rejected the idea at different points in its history, leading to inconsistent policies across federal agencies. Some agencies are banned from using QALYs to make benefits and coverage decisions, while others use them frequently.
- There has been increasing interest by the Federal Government in reducing the cost of health care by modeling parts of its national health insurance programs after the healthcare systems of other countries, such as the United Kingdom. Several of these countries utilize QALYs to make benefits and coverage decisions. The coverage denials and loss of access to care faced by people with disabilities in these countries illustrate what might happen if the United States made a similar choice.
- QALYs and cost-effectiveness research are one of many different types of evidence insurers consider when making their decisions. There is limited publicly available evidence that shows to what extent private health insurance companies use QALYs and cost-effectiveness research to inform their medicine and medical treatment-related decision making.

NCD has found sufficient evidence of QALYs being discriminatory (or potentially discriminatory) to warrant concern . . .

QALYs and the analyses that rely on them are most likely utilized in insurers' internal decision-making processes, for which there is little transparency.

- There are alternatives to the use of QALYs. These alternatives range from well-established methods regularly used by United States federal agencies already, such as cost-benefit analysis, to unexplored but promising alternatives such as value frameworks that use patient preferences to determine the value of healthcare treatments. Many alternatives may themselves be discriminatory if used in certain contexts, or if they are used without paying sufficient attention to the possibility that discrimination may occur. However, several (such as multi-criteria decision analysis [MCDA], which allows its user to consider multiple unrelated benefits of a treatment and weight each benefit individually before arriving at a decision) can be used in a nondiscriminatory manner. It is much more difficult, if not impossible, to use QALYs in a nondiscriminatory manner. No single alternative serves all of the functions of QALYs.

QALYs and the analyses that rely on them are most likely utilized in insurers' internal decision-making processes, for which there is little transparency.

- Avoid creating provisions of any bill that would require the agency with management and oversight responsibilities (such as, for example, HHS) to cover only the most cost-effective drugs and treatments, or to require the agency to impose restrictions on less cost-effective treatments.

Congress should pass legislation:

- Prohibiting the use of QALYs by Medicaid and Medicare.
- Provide funding to Health and Human Services (HHS) for research on best practices on the use of cost-effectiveness to inform benefits and coverage decisions with respect to US national health insurance programs, such as Medicare and Medicaid. "Best practices" in this case refers to a means of utilizing cost-effectiveness research that facilitates greater access to care, and does not reduce access to care for people with chronic health conditions and disabilities.

US Department of Health and Human Services (HHS), Office for Civil Rights (OCR); US Department of Justice (DOJ) Civil Rights Division

DOJ and OCR should jointly issue guidance clarifying that the ADA applies to coverage programs that states operate such as Medicaid.

OCR, in consultation with DOJ as appropriate, should issue guidance to HHS sub-agencies, such as the Centers for Medicare and Medicaid Services (CMS) as well as to state Medicaid agencies, clarifying that:

Key Recommendations

Congress

When enacting health reform bills, Congress should:

Section 504 and Section 1557 also apply to Medicaid programs because they receive federal financial assistance. The guidance should specifically discuss how these authorities apply to benefits and reimbursement decisions, and that payment decisions should not rely on cost-effectiveness research or reports that are developed using QALYs.

Section 504 and Section 1557 apply to health insurance programs operated by recipients of federal financial assistance from HHS. The guidance should discuss that covered health insurance programs should not rely on cost-effectiveness research or reports that gather input from the public on health preferences that do not include the input of people with disabilities and chronic illnesses.

HHS

- HHS should consider including explicitly recruited people with disabilities and chronic illnesses as members of committees and working groups formed to develop effective healthcare reform and strategies for lowering the cost of prescription drugs.
- HHS should support healthcare providers by issuing guidance on what steps to take if their patient's health insurance agency refuses to cover recommended treatment on the basis of that treatment's cost-effectiveness.

HHS, OCR

- OCR should issue guidance to HHS sub-agencies, such as Centers for Medicare

and Medicaid Services, State Medicaid Agencies, clarifying that:

- Title II of the Americans with Disabilities Act (ADA) applies to national health insurance programs jointly run by the Federal Government and the States, such as Medicaid. The guidance should specifically discuss how the ADA applies to benefits and reimbursement decisions, and that payment decisions should not rely on cost-effectiveness research or reports that are developed using QALYs; and
- Insurance programs jointly run by the Federal Government and the States, such as Medicaid, should not rely on cost-effectiveness research or reports that gather input from the public on health preferences that do not include the input of people with disabilities and chronic illnesses.

HHS, CMS

- CMS should utilize well-established alternatives to QALYs, such as MCDA, which is a method that better acknowledges the complexity of healthcare coverage decisions, or cost-benefit analysis, when the exact benefits and costs of a drug or treatment are known. CMS could utilize these methods in combination, such as using cost-benefit analysis as one component of an MCDA. If CMS does utilize cost-effectiveness analysis, it should consider utilizing it as one component of a condition-specific MCDA.

Acronym Glossary

ADA	Americans with Disabilities Act
ASAN	Autistic Self-Advocacy Network
CBO	Congressional Budget Office
CDC	Centers for Disease Control and Prevention
CEA	cost-effectiveness analysis
CMS	Centers for Medicare and Medicaid Services
CUA	cost-utility analysis
DOJ	US Department of Justice
DREDF	Disability Rights Education & Defense Fund
evLYG	equal value of life years gained
FDA	US Food and Drug Administration
GDP	gross domestic product
HHS	Health and Human Services
HTA	health technology assessment
ICER	Institute for Clinical and Economic Review
IPI	International Pricing Index
ISPOR	International Society for Pharmacoeconomics and Outcomes Research
MCDA	multi-criteria decision analysis
NCD	National Council on Disability
NHS	National Health Service
NICE	National Institute for Health and Care Excellence
OCR	Office for Civil Rights
PBM	pharmacy benefit managers
PCORI	Patient Centered Outcomes Research Institute
PIPC	Partnership to Improve Patient Care
PPVF	Patient Perspective Value Framework
QALY	quality-adjusted life years
VA	Department of Veterans Affairs

Some stakeholders, but especially bioethicists and people with disabilities, have argued that QALYs are built on a faulty premise: that life with a disability is inherently worse than life without a disability.



Introduction

Healthcare spending has become a major concern in policy discussions across the United States. Concern is growing in large part due to the rapidly rising cost of health care. In 1973, healthcare spending amounted to 7.5 percent of US gross domestic product (GDP), while in 2017, healthcare spending more than doubled to approximately 18 percent of US GDP.¹

In 1973, the United States spent just \$102.8 billion dollars² on health care, while in 2017 total US healthcare spending had risen to nearly 3.5 trillion dollars.³ In this context, policymakers have rightly sought various means of lowering total healthcare costs.

One of the major means that has been considered by healthcare policymakers (such as US federal agencies, health economists, etc.) is the idea of health insurers and other payers funding “high-value” treatments over “low-value” treatments.⁴ Patients and payers may significantly differ in how they interpret which treatments are of “high value” to them. For many payers, however, a high-value drug

or treatment is merely a cost-effective one. A cost-effective treatment is a treatment that significantly extends life or improves patient quality of life (or both), at a cost which, to the

payer, does not outweigh the improvements to health it provides. Payers may rely on a variety of evidence to determine cost-effectiveness, particularly cost-effectiveness analysis (CEA) studies, which

examine the cost-effectiveness of drugs and treatments.

Several nonprofit organizations and professional associations in the United States have also attempted to help payers determine

which treatments are of the highest value. To this end, they have created decision-making tools known as value frameworks, many of which primarily focus on

cost-effectiveness.⁵ Value frameworks can be used to produce reports that evaluate new drugs and treatments (sometimes known as health technology assessment reports, or HTAs).⁶ The most influential of these HTAs are produced by

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Patients and payers may significantly differ in how they interpret which treatments are of “high value” to them.

the Institute for Clinical and Economic Review (ICER), whose reports are relied on by payers as varied as the pharmacy benefit manager CVS Caremark and the Veterans Administration.

In prioritizing cost-effective treatments and treating cost-effectiveness as identical to value, however, payers may risk using means of quantifying which treatments are cost-effective that are simplistic and potentially discriminatory, such as QALYs.

QALYs are a measure that attempts to show the extent to which a particular treatment extends life and improves quality of life at the same time. QALYs are an important outcome measure in several influential value frameworks, such as ICER's value framework. QALYs are also used extensively to make healthcare coverage and reimbursement decisions in other countries. For example, the National Institute for Health and Care Excellence (NICE) in the United Kingdom uses QALYs when determining what Britain and Wales' single-payer healthcare system, the National Health Service (NHS), will cover. Health outcomes for some patients with chronic illnesses and disabilities (such as patients with lung cancer) are notably worse in the United Kingdom than in the United States.⁷

Many stakeholders are therefore concerned that the way QALYs are calculated devalues treatments that extend the lives of people with disabilities, or treatments that mitigate—without eliminating—the impact of disability on their health. They argue that if value frameworks that use QALYs become more influential, people with disabilities will lose access to needed care. Other stakeholders view QALYs as a way to provide necessary information on the benefits and costs of healthcare in a healthcare system

that has been put under strain by rising costs. This report examines how QALYs are calculated, the bioethical implications of using QALYs, and the history of the use of QALYs in the United States.

Summary of Methodology

In order to get a clear and comprehensive picture of the use of QALYs in the United States, the NCD research team consulted bioethicists, patient rights advocates, researchers and health economists, people with disabilities and their families, and relevant scholarly articles from bioethical, economic, insurance agency, and healthcare system perspectives.

Qualitative Data

To understand how the quality-adjusted life year was used by payers and to better inform the conclusions reached, NCD conducted seven in-depth interviews with disability rights advocates, representatives of advocacy organizations who serve patients, two bioethicists with a significant understanding of the ethical issues presented by QALYs, a representative of an organization that reviews value frameworks to determine their degree of patient-centeredness, and a representative of the nonprofit Institute for Clinical and Economic Review, which uses QALYs. Additionally, the research team conducted a stakeholder convening on September 24, 2018 to inform and aid NCD in the initial development of this report.

Literature Review

To obtain information on how QALYs are used, as well as the perspectives and opinions of ethical experts and experts in the field of health

economics on its use, NCD reviewed articles from research journals, bioethics journals, and news articles pertaining to the use of the quality-adjusted life year. NCD also conducted an in-depth review of several value frameworks,

including FasterCures' Patient Perspective Value Framework, ICER's Value Assessment Framework, and the condition-specific decision-making tools created by the Innovation and Value Initiative.

Chapter 1: How QALYs Are Calculated and the Impact on People with Disabilities and Patients with Chronic and/or Degenerative Illnesses

The Purpose of QALYs

In order to understand how to calculate QALYs, it is important to explain both what QALYs are supposed to represent, and why they are used.

What QALYs Represent

Normally, when a researcher or scientist tries to determine whether or not a healthcare treatment (like chemotherapy) improves health, they are looking at one of two different things:

- whether the treatment extends the patient's life, or
- whether the treatment improves the quality of the patient's life.⁸

While measuring whether or not a treatment extends life is fairly straightforward, measuring the degree to which a treatment improves someone's quality of life is more complicated. The portion of a person's quality of life that relates to their health is called their health-related quality of life.⁹

Health-related quality of life is a broad concept. According to the Centers for Disease Control and Prevention (CDC), at the individual level, it may include a person's mood and energy levels, their physical and mental health, and the elements of the person's life that contribute to these factors—such as some aspects of the person's disabilities, health risks,

and their social and socioeconomic status.

If measured at the population level, it includes any "conditions, policies, and practices that influence a population's health perceptions and functional status."¹⁰ Health researchers and government agencies (including the CDC itself, by conducting population-level surveys using a set of 14 questions called "Healthy Days Measures")¹¹ have created different means of measuring health-related quality of life.

When healthcare payers decide how to spend their money, they are often looking for some way to represent all the benefits a particular treatment provides at once, as this saves them time. However, studies of treatments tend to measure benefits of treatment that are qualitatively different from one another, such as life extension and quality of life, separately from one another. For example, a study could measure the length of time a patient survives after treatment, or the number of days the person is free from pain, but perhaps not both in the same study.¹² It may be difficult, therefore, to directly compare the value of a treatment that primarily extends life to the value of a treatment that primarily improves quality of life.¹³

QALYs are one attempt to get around this problem. QALYs are the product of an equation designed to "combin[e] the effects of health interventions [treatments] on morbidity [quality

of life] and mortality [quantity of life] into a single index.”¹⁴ The QALY equation does this in a rather simplistic fashion. It simply lowers the value of a year of treatment by the degree to which an illness or disability is perceived to harm the person’s quality of life during that year.¹⁵ QALYs typically are calculated before and after treatment to determine the degree to which a treatment improves the number of QALYs gained by the patients being studied.¹⁶

QALYs are calculated by multiplying a decimal number between 0 and 1, which represents a person’s health-related quality of life, by a number representing quantity of life. The “quantity” can be the number of years by which the treatment extends life, the number of years a person expects to have to take the treatment, the amount of time a person has left to live, or any other time period relevant to the researcher. A typical QALY calculation is shown in the “QALY Calculation” box.

Ari Ne’eman, a disability rights advocate and expert on QALYs, described what QALYs are and what they do in this way:

The QALY works by weighting the lives of people with disabilities: If we were to assign autism a disability weight of 0.2, that [number] would mean that a year in the life of an autistic person would be worth 80 percent of a nondisabled person’s life. Different disabilities would get a different number, if

you assigned 0.5 to a mobility impairment, then a year in that person’s life would equal 50 percent of a nondisabled life year.

A flowchart showing how QALYs would be calculated if the researcher or scientist used a commonly utilized questionnaire—the EQ-5D—is included as Appendix A of this report.

Why QALYs Are Used

Why would it be *necessary* to measure both quantity of life and health-related quality of life at the same time? The most frequently provided explanations in research literature for the use of

QALYs are: (1) to compare the impact of multiple treatments for unrelated conditions to one another; or (2) to assess whether a new treatment or drug would be more cost-effective than the drug or treatment that is currently being used.¹⁷

This report focuses on the most common use of QALYs: their use by

health economists, researchers, and nonprofits to perform cost-effectiveness analyses (CEAs) and health technology assessments (HTAs); the

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QALY Calculation

Number between 0 and 1 representing quality of life of x number of years = number of QALYs

subsequent use of CEAs and HTAs by private and public health insurers to determine what drugs or treatments they will fund; and the real and potential negative impact CEAs and HTAs have on people with chronic illnesses and disabilities' access to physician-recommended drugs and treatments.

Cost-Effectiveness Studies

Cost-effectiveness studies are designed to compare various healthcare treatments to each other and determine whether the benefits of a healthcare treatment are worth the treatment's cost. The type of cost-effectiveness study that uses QALYs is called a cost-utility analysis (CUA).¹⁸ In a CUA, the number of QALYs gained from treatment is a measure of the "health outcome," or the overall benefit of the treatment.

The difference between the cost-effectiveness of the treatment being examined and another treatment being examined by the researcher (typically, the treatment currently in use) is referred to as the treatment's incremental cost-effectiveness ratio, or "ICER."¹⁹ The ICER is often used when comparing the cost-effectiveness of multiple treatments.²⁰ When using QALYs, the ICER is often referred to as the treatment's "cost per QALY," although it is possible to get the "cost per QALY" of a single treatment.²¹ At its most simple, it is important to know that the lower the cost per QALY, the more cost-effective the treatment is considered to be.

QALYs are also used in some of the decision-making tools known as "value frameworks." When QALYs are used in a value framework, it is typically because CUA studies are used as evidence of the benefits and costs of the treatment being evaluated by the report. Use of the report can mean that, instead of having to weigh any number of complex considerations relating to whether or not a treatment should be covered, payers can simply fund the treatment that has a better "cost per QALY," according to its corresponding report. CUAs and other QALY-based reports and research studies are *not* healthcare policies in and of themselves, but rather are used to

inform the development of healthcare policies (for example, insurers' drug formularies).

Calculation of Quality-Adjusted Life Years

While the equation used to calculate QALYs is always the same, there is no one single way to calculate the numbers that go into that equation. For instance, there are many different ways to calculate the number between 0 and 1—often called the "health utility"—that represents health-related quality of life. However, there are common methods typically used by many health economists and researchers employing QALYs in CUA studies. Many components often used to calculate QALYs are used internationally. The EQ-5D,²² a questionnaire frequently used to calculate QALYs, is used in countries as diverse as the United Kingdom,²³ Iran,²⁴ and China.²⁵

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Health Utilities

To calculate a QALY, it is necessary to determine *by how much* not being in perfect health impacts a person's quality of life. QALYS do this by assigning a number between 0 and 1, called a health utility, to the various conditions a person's health could be in (often called "health states").²⁶ A 0 would represent the lowest possible quality of life, while a 1 would represent the highest possible quality of life. Health states are represented by points on the scale of 0 to 1—for example, 0.2, 0.5, 0.8.

Health utilities are typically derived from surveys, which attempt to determine how much survey participants would prefer to be in one health state as compared to another. Health states do not correspond directly to specific disabilities—they instead represent the degree of impairment a person has in specific, limited categories of functioning (such as mobility, ability to perform tasks, etc.). However, most disabilities share some or all characteristics of a health state. Therefore, the goal of a "health utility" is, in effect, to measure the degree to which having a particular form of a disease or disability, such as "having late-stage cancer" or "having a specific type or degree of type 2 diabetes," is viewed as negatively impacting quality of life as compared to a state of perfect health.²⁷

Questionnaires Used to "Describe" the Health State, and Their Flaws

As noted above, the first thing the researcher has to do is determine how having a disability

or illness impacts a person. Typically, in order to obtain this information, the researcher has a sample of patients with the illness, condition, or disability fill out a survey or questionnaire.²⁸ There is no one, single definitive questionnaire or survey that is used.²⁹ The most common questionnaire is the EQ-5D.³⁰ The EQ-5D is extremely popular internationally.³¹

The EQ-5D takes an extremely limited approach to measuring "quality of life." Use of the EQ-5D requires patients to rate the degree to which they have "problems" with only a few extremely broad categories of "physical, cognitive, or social functioning," rather than the myriad of effects someone's health could have on their quality of life.

The EQ-5D surveys patients' health as it relates to five "dimensions" of quality of life: mobility, self-care, usual activities, pain/discomfort, and anxiety/depression.³² These five categories do not measure the wide variety of impacts a disability or illness could have on quality of life. NCD interviewed the bioethicist Joseph Stramondo, who said "I think that, while there is a relationship between disability and quality of life, it is extremely variable, and impossible to generalize. There are all kinds of things [about disability and illness] that impact quality of life on a case-by-case basis: relationships, income, accessibility considerations." Moreover, neither "self-care" nor "usual activities" are defined in detail anywhere in the sample questionnaires available

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on the EQ-5D website, meaning that many patients may not know what these terms mean for them. Furthermore, there is no way to account for external factors, like the availability of reasonable accommodations or the accessibility of the built environment, as a factor in the assessment of quality of life with a disability, despite the fact that these factors play a significant role in determining the life experience of many people with disabilities.

Impacts on these dimensions are then rated by “severity.” Different forms of the EQ-5D exist. The oldest and most commonly used form, the EQ-5D-3L,³³ assigns three “levels of severity” to each of the five dimensions. For each dimension, it is possible for the person taking the survey to respond “I have no problems,” “I have some problems,” or “I have extreme problems.”³⁴ For example, the EQ-5D-3L User Guide includes the following sample question on mobility:

Questions Asked on the EQ-5D-3L Questionnaire

Mobility

- I have no problems in walking about
- I have some problems in walking about
- I am confined to bed

Note that this question is focused on whether a person has problems “walking about,” and the most severe problems are described as the person “being confined to bed.” The questions do not appear to consider the possibility that a person who cannot “walk about” can still move, such as a person who cannot walk but who can use a wheelchair.

Nor does the EQ-5D consider the possibility that a person who can walk may nevertheless have significant trouble leaving the home due

to other concerns, such as the need to stay near medical equipment, concerns about exposure to infections, or agoraphobia.

As noted by Stramondo and a colleague in an article on disability and its relationship to quality of life, impairment in performing a specific

task may have no relationship to quality of life.³⁵ The questionnaire assumes that a person will experience difficulty with walking as a significant barrier to subjective quality of life when, in fact, this is not true of many people with mobility impairments. Although there are several versions of the EQ-5D, and other versions do not phrase the question and/or questions in this manner, the other versions also assume that being unable to walk has a severely negative impact on quality of life.³⁶

In the EQ-5D-3L, each dimension receives a score from 1 to 3, where one is the best possible score and 3 is the worst possible score. Thus, a person who checked the first box, “I have no

“I think that, while there is a relationship between disability and quality of life, it is extremely variable, and impossible to generalize. There are all kinds of things [about disability and illness] that impact quality of life on a case-by-case basis: relationships, income, accessibility considerations.”

problems,” would be assigned a score of 1 for mobility.³⁷ Filling out the entire questionnaire generates a series of five numbers, each of which is between 1 and 3. For example, a score of 11111 means the person is in perfect health, whereas a score of 11223 means the person has no problems with the first two dimensions, some problems with the next two, and extreme problems with the final dimension.³⁸

When using the EQ-5D-3L to calculate QALYs, it is this series of five numbers which was actually evaluated, as opposed to the actual disability and the actual effect of the disability on physical or psychological functioning as reported by people with that disability. The people who decided the value of life with a particular condition only saw those five numbers and/or a description of what those numbers meant.³⁹

Aside from the dehumanizing implications of disability’s impact on quality of life being reduced to a series of five numbers, if two different disabilities had exactly the same impact on physical or psychological functioning, they would have exactly the same health utility value for the purpose of calculating QALYs—even if they had other differences that some people may consider relevant to “quality of life.” The numbers are based only on the disability or illness’ impact on “physical, psychological, cognitive, social or other kinds of functioning,”⁴⁰ as defined by the survey.

Patients with two conditions with the same utility value may have very different opinions about which aspects of their conditions are most important to address, and what kinds of treatments would most improve their lives. Nonetheless, treatments that improved their health utility scores to the same degree would be treated as having the exact same value to the patients. For example, patients with Disability A could place a higher value on reducing pain and a lower value on reducing anxiety and depression.

Patients with Disability B could place a lower value on reducing pain and a higher value on reducing anxiety and depression. If patients with these disabilities received the same average EQ-5D score, a treatment that reduced pain would be treated as if patients with Disability B valued it to the same degree as patients with Disability A.

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Most other questionnaires share similar issues. For example, the SF-6D looks at the impact of an illness or disability on “physical functioning,” the degree to which one’s emotional problems limit their ability to perform daily tasks, and so on, and uses specific, narrow questions to determine the impact.⁴¹ Additionally, using different questionnaires results in different numbers of QALYs, which raises validity and reliability concerns, when different methods produce results that are not comparable.⁴²

The validity of these generic questionnaires can be called even further into question by the

fact that the utility values are often not calculated separately for each individual cost-effectiveness study. Instead, the utility values are often based on the outcome of specific past studies in which members of the general public valued a sample of the possible health states (with values for the health states not valued determined mathematically from the values of the health states that were valued).⁴³ The EuroQoL group, maker of the EQ-5D, refers to these studies as “value sets.”⁴⁴

Valuation of Disability

Regardless of how the impact is assessed, once the researcher assesses the impact of a health condition on health, the researcher needs to determine how much “worse” it is to be in that condition as compared to perfect health. This is done by determining the degree to which a group of people would prefer to be in that health state as compared to perfect health.

The researcher can either measure the preferences of patients with the disability or measure the preferences of the general population.⁴⁵ While there are those in the field that advocate for using “patient preferences”⁴⁶ and those who advocate for using “population preferences,”⁴⁷ the overwhelming majority of studies use the preferences of members of the general population (76 percent, according to one study).⁴⁸

The preferences of the general population are typically calculated by surveying a sample of the general public and asking them a series of

questions. Researchers performing a CUA ask a person to imagine a hypothetical situation and respond to questions about that hypothetical situation. There are two types of questions researchers typically ask the public: Time Trade-Off questions and Standard Gamble (SG) questions.⁴⁹

In a Time Trade-Off, survey participants are asked to determine how many years of living with a particular disability (for example, 70 years of blindness) they would trade for a shorter number of years spent in perfect health (for

example, 50 years of perfect health).⁵⁰ In a Standard Gamble, the participants are asked to imagine having a disability and then are asked whether they would undergo a procedure that had, for example, a 50 percent

chance of returning them to perfect health and a 20 percent chance of instantly killing them.⁵¹

If members of the public respond in a way that suggests that they see 20 years in a health state corresponding to a specific type of blindness and 17 years of perfect health as having the same value, the researcher will divide 17 by 20 to get a health utility value of 0.85 for the health state corresponding to that specific type of blindness.⁵²

Many would contend that members of the general public do not accurately understand the experience of life with a disability and will systematically underestimate the value of disabled quality of life. However, surveying people with disabilities poses other problems. Since people with disabilities tend to rate their quality of life as higher than the perception of it from the

[U]sing different questionnaires results in different numbers of QALYs, which raises validity and reliability concerns, when different methods produce results that are not comparable.

general public, leading to lower health utilities, the use of survey responses from people with disabilities will increase the value of life-extension while reducing the value of quality of life improvements. Because the QALY compresses these two factors into a single number, it forces a choice between prioritizing life extension and quality of life improvement. In one article, bioethicists referred to QALYs' inability to simultaneously value treatments that extend and improve the lives of people with disabilities as "the QALY trap."⁵³ According to Ne'eman, this problem can be substantially mitigated or eliminated by using diagnosis- or domain-specific measures, such as lung function, pain scales, or functional skills, since these do not conflate morbidity and mortality into a single number. As Ne'eman stated in his interview with NCD:

If you go with a system [for calculating QALYs] that surveys the general public, you are likely to end up with more resources willing to be spent on disability or disease mitigation. If you survey [people with disabilities], you're likely going to end up with more going to life extension. But it forces you to choose. Then you should ask—is this a good system?

This speaks to one of the fundamental flaws of the QALY: that the conflation of life extension and quality of life improvement benefits into a single number forces people with disabilities into a cruel trap: picking whether they would rather live longer or have improved quality of life, when

both are entirely feasible in a society willing to invest sufficient resources.

Dr. Steve Pearson, bioethicist at the National Institutes of Health and the President of ICER, agrees that surveying only people with the condition is problematic, but surveying the healthy community is also problematic unless they are informed about the conditions they are judging. According to Dr. Pearson,

In order to get the best information, they [the healthy community] need to know what it is like to live with that condition. You want to know if their opinion on how bad

something is, is higher or lower or the same as the person who actually does have the condition. . . . Maybe the healthy person, with no knowledge of the condition,

would think the opposite of the person with the condition. Maybe they think it is not so bad having psoriasis, maybe it's a skin rash that's not so bad. But then you talk to a person with psoriasis and they say, "It's awful—you never want to have this! It's painful—you have no idea." . . . Though there are hypothetical and ethical reasons people tend to still use the healthy community, it still should be informed by the patients.⁵⁴

David Wasserman, a bioethicist at the National Institutes of Health, also agrees with the limitation of surveying only people with the conditions, but believes that surveying the healthy community, even when they are provided

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with information about the condition, is not effective. According to Wasserman,

Public opinion is extremely labile. It's influenced by a lot of factors like media presentation, exaggerated optimism, occasionally by excessive skepticism, by poignant anecdotes . . . so, I don't think that you can generally trust popular judgments. Even carefully elicited popular judgments have serious problems. One approach is that we should rely on the preferences of the general public about health states, but the general public doesn't have the health states in question, so let's give them information on how people in those health states regard them. But even if you give them that information, they will almost surely disregard it. . . .⁵⁵

Calculating QALYs

The method for calculating QALYs is best expressed using an example. This example additionally demonstrates one of the primary ethical objections to the use of QALYs.

Example 1: Connie and Bill

Connie has a disability. People with Connie's disability have difficulty performing daily living tasks and lose the ability to walk. Connie now uses a wheelchair for mobility, as do most people with Connie's disability. Without treatment, people with Connie's disability have 4 years left to live after they are diagnosed. Based on the responses of patients with Connie's disability to the EQ-5D, researchers have calculated the health utility, or value of a life with Connie's problems with daily living and need for a wheelchair, as 0.5. To get the number of

quality-adjusted life years she would get from living for 4 years with her disability, one must use the following equation:

$$0.5 \text{ (health utility)} \times 4 \text{ (the number of years Connie has left to live)} = 2 \text{ QALYs}$$

Thus, the 4 years people with Connie's disability are expected to live without treatment would be valued at only 2 QALYs.

A drug that is found that would extend the life of people with Connie's disability by 20 years, but it would not remove or reduce the impact of the disability on daily living; they would still use wheelchairs. The health utility of their condition is still 0.5. Thus, Connie's life expectancy with treatment is valued at 10 QALYs. This can be expressed via the following equation:

$$0.5 \text{ (utility value)} \times 20 \text{ (the number of years Connie would have to live if the treatment for patients with her disability was covered)} = 10 \text{ QALYs}$$

If people with Connie's disability were the only patient demographic that needed health care, the treatment that people with Connie's disability needed would probably be considered cost-effective for the insurer because these individuals would gain 8 QALYs from being treated.

However, there is another patient, Bill. Bill has a medical condition that also has a health utility of 0.5 and that causes patients with that disability to need a wheelchair. Patients with Bill's disability will only live for another 4 years without treatment, and would also gain only 2 QALYs during those 4 years without treatment.

There is a drug that would extend the lives of these patients to 20 years, but would also raise their quality of life back up to 1—the utility value for “perfect health.” This would mean that Bill and other patients with his disability would no longer have difficulty with daily living tasks and no longer need a wheelchair. This can be shown using the following equation:

$$1 \text{ (health utility)} \times 20 \text{ (the number of years Bill could live if the treatment for patients with his disability was covered)} = 20 \text{ QALYs}$$

Given that patients with Bill’s condition will gain 18 QALYs from being treated as compared to patients with Connie’s condition, who would only gain 8 QALYs, the drug for patients with Bill’s condition will be considered more cost-effective than the drug for patients with Connie’s condition. For the purposes of this example, the two treatments cost exactly the same amount of money, and the payer only has enough money to pay for one of these two treatments at this time. If the payer relies on QALYs to determine how cost-effective the two drugs are, the payer will favor covering the treatment patients with Bill’s disability need over the treatment patients with Connie’s disability need.

In an environment with scarce resources, Bill’s condition will be more likely to have treatments for it funded than Connie’s. While these decisions are typically made at the population level, rather than in relation to specific patients, they create an environment of systemic inequality, where people with disabilities and chronic conditions that will be managed, rather than cured, are less likely to receive access to treatment under health systems that ration care utilizing the QALY.

Calculating Cost per QALY

When trying to decide whether to cover a treatment, most payers are interested in the “incremental cost-effectiveness ratio,” which is typically the difference between the cost-effectiveness of the treatment that is being studied as compared to another treatment (which is often either another possible treatment for the same illness or problem, a placebo, or the standard therapy that is currently in use).⁵⁶ In the box “Cost per QALY,” “ICER” stands for incremental cost-effectiveness ratio. As explained above, the ICER is often referred to as the “cost per QALY,” although the cost per QALY of a single treatment can theoretically be calculated. One can calculate the ICER by using this formula.⁵⁷

Cost per QALY/ICER

$$\text{ICER} = \frac{(C1 - C0)}{(E1 - E0)}$$

In this formula, C means “Cost,” C1 represents the treatment being studied, and C0 represents either the current treatment or another treatment being considered for coverage. E means “Effect,” E1 represents the number of QALYs gained from the treatment being studied, and E0 represents the number of QALYs gained from either the current treatment or another treatment being considered for coverage. To obtain the “cost per QALY” of a single treatment rather than an ICER (although this is less common), divide the treatment’s cost by the number of QALYs gained from treatment.

Some payers have a specific threshold cost-per-QALY. For example, a payer could decide that they will not cover any treatment that costs more than \$50,000 per QALY.

Methodological Flaws of Quality-Adjusted Life Years

QALY calculations are subject to several methodological flaws that seriously undermine their use as a fair method of comparing the relative value of treatments.

QALYs Do Not Fully Measure Health-Related Quality of Life

One significant flaw of QALYs is simply that they do not measure what their proponents claim they measure:

the combined impact a treatment has on life expectancy and quality of life. As discussed in the section “Questionnaires Used to ‘Describe’

the Disability and Their Flaws,” the generic, population preference-based questionnaires often used to calculate QALYs only measure a few specific impacts of health on quality of life, such as pain or anxiety/depression, and may not measure these accurately and in a way that fully considers the possible accommodations available to a person with a disability. This means that QALYs undervalue treatments that affect aspects of quality of life other than what they specifically measure. For example, many people with psychiatric disabilities report significant side effects associated with certain medications, like tardive dyskinesia or weight gain. QALY calculations might not value medications that allow people with disabilities to avoid these

side effects, since they focus only on measures surrounding the mitigation of the primary condition rather than the complex context surrounding that individual’s life.

Similarly, the level of quality of life experienced by a person with a disability or patient may shift dramatically based on nonhealth factors, such as the availability of reasonable accommodations or the accessibility of the built environment. For example, the impact of a mobility impairment on quality of life is significantly altered based on the availability of a wheelchair and a built environment that encompasses ramps. Similarly, the impact of a cognitive disability is significantly altered based on the availability and quality of special education services. Typically, the use of QALY assessments

in healthcare contexts do not consider these factors, which may play an equal or greater role in quality of life than a purely medical assessment. Additionally,

the utility values used to describe the extent to which a disability impacts quality of life are derived from people without disabilities, who often have prejudices and biases that lead them to drastically undervalue life with a disability.

Palliative Care

Failure to consider all aspects of quality of life, combined with the weighting of quantity and quality of life simultaneously, may lead QALYs to undervalue treatments that are purely palliative in nature. The main purpose of palliative care is to alleviate the pain and suffering of a person who has a serious and/or life-threatening illness. Often, these illnesses are expected to lead to death, as in the case of late-stage cancer or kidney

disease.⁵⁸ Palliative care may include treating pain, fatigue, reducing the difficulty the person has sleeping, or reducing the amount of anxiety and depression experienced by the person.⁵⁹

The first problem is simply that palliative care patients often are not expected to live for many more years. Since QALYs measure both quality *and* quantity of life in the aggregate, and palliative care rarely improves a patient's life expectancy, a patient cannot expect to gain many QALYs from a palliative care treatment.⁶⁰

The second problem is that there are things that are very important to palliative care patients' evaluation of their own quality of life—such as spiritual contentment and personal dignity—that are rarely if ever measured by the generic questionnaires (such as the EQ-5D) used to calculate QALYs.⁶¹ This may mean that palliative care is undervalued as compared to other treatments.

Finally, QALYs assume that the value of a year of life to the patient is the same regardless of when that year is lived, which most studies have found is simply not true, from the patient's perspective. Patients with a limited number of years left to live typically value a year much more highly than people who have many more years left to live.

Dr. Steve Pearson disagrees with the concern that, due to their design, QALYs may undervalue palliative care treatments and treatments that mitigate the impact of a disabling condition, but do not cure it or extend the patient's life. Pearson told NCD that the QALY would do exactly the opposite, and that,

We [the Institute for Clinical and Economic Review] did a cost-effectiveness analysis of outpatient palliative care that showed it was cost-saving. When something is cost-saving you don't do cost-effectiveness analysis per se, but the thing about palliative care is that it improves quality of life without extending life—although some palliative care does, and the sicker you are the better that will look, in some sense, because if you are already quite well there's not much to palliate . . . the QALY was built to capture improvement in quality of life of that type.

Pearson thinks that "the question is which is the more cost-effective way to provide pain

Failure to consider all aspects of quality of life, combined with the weighting of quantity and quality of life simultaneously, may lead QALYs to undervalue treatments that are purely palliative in nature.

control for [people who are] dying, not whether we [as a society] should or shouldn't."⁶² This may be the case if the cost-effectiveness of palliative care treatments were being compared to hospitalization (or another

high-cost, low-value treatment for the patients who typically utilize palliative care) or only to other palliative care treatments. It is, however, difficult to know if this would be true if palliative care treatments were competing with other uses of the same funds, at the budgeting level.⁶³ Even researchers who support the use of QALYs in palliative care note that "the brevity of lifespan affected results in palliative care yielding a fraction of a QALY unit," and that the use of QALYs to help allocate healthcare funding means that new palliative care treatments are always competing with alternative uses of the same money.⁶⁴ While payers are not attempting to

determine whether pain care for the dying is “worth it,” they may be attempting to determine whether *improving* pain care is, as compared to some other use of their limited funds.

Additionally, researchers who are interested in utilizing QALYs for palliative care typically propose modifying the standard QALY, either by using palliative care-specific questionnaires that do evaluate the quality of life aspects most important to palliative care patients or by incorporating their higher valuation of time spent at the end of life into the calculation.⁶⁵ Other researchers propose only comparing end-of-life treatments to other end-of-life treatments.⁶⁶

The need to modify the standard QALY to work for palliative care indicates that QALYs are unsuitable without modifications. There are likely many other specific diseases and circumstances for which the use of QALYs is unsuitable without modifications, which undermines the claims of those who state that QALYs are a metric that can be used to compare the value of treatments for unrelated conditions.

When Health Utilities Are “Zero”

QALYs could produce problematic results if a treatment extends the life but does not significantly improve the “quality of life” (as measured by QALYs), of a patient whose life’s worth has been measured as 0, close to 0, or less than 0. In these cases, even the cheapest treatments to extend life would not be considered “cost-effective” according to a cost-per-QALY standard.

When Health Utilities Are Less Than 0

Patients with Life-Threatening Condition Y fill out the EQ-5D questionnaire and get a score of 33333. Solely in this example, members of the general population who performed a Time Trade Off decided that the utility value of this health state (and by extension, therefore, Life-Threatening Condition Y) was 0. Treatment 1 would extend the lives of patients with Life-Threatening Condition Y by a year. However, the following simple equation illustrates that these patients would nonetheless obtain 0 QALYs:

$$0 \text{ (health utility)} \times 1 \text{ (number of years by which Treatment 1 extends their life)} = 0 \text{ QALYs}$$

There are likely many other specific diseases and circumstances for which the use of QALYs is unsuitable without modifications, which undermines the claims of those who state that QALYs are a metric that can be used to compare the value of treatments for unrelated conditions.

This is due to the way that QALYs aggregate quality of life and quantity of life. “When Health Utilities Are Less Than 0” explains how this can happen in more detail.

For example, if the health utility of having a particular disease or disability is measured as 0 or negative, it may

inevitably lead to the conclusion that the person is “better off dead” and that treatments that prolong such a life are not cost-effective.⁶⁷ Such an outcome would only be acceptable if a person were in a health state in which everyone

would agree that continued life has no value. However, as the bioethicist Stephen Barrie noted, the meaning of the “zero” on the health utility scale is ambiguous and patients do *not* always agree that continued life in a health state that earns very low or even 0 QALYs has no value. A score of 0 QALYs has meant “being dead,” “dying,” and “worst possible health state,” depending on the study and who was doing the calculating—and these are three very different things.⁶⁸ Some individuals may believe “dying” is worse than “being dead.” Some people with a health state that has been judged to be the “worst possible” may wish to discontinue treatment, while others may still highly value an additional year of life. QALYs do not make these distinctions—researchers using them would need to treat all three health states as equally valueless.

Distinguishing Between Subgroups of Patients with the Same Condition

Some individuals argue that QALYs do not distinguish between subgroups of patients with the same condition. Subgroups of patients include but are not limited to patients of different races/ethnicities, patients with different genders or ages, and patients with other co-occurring illnesses.⁶⁹

A score of 0 QALYs has meant “being dead,” “dying,” and “worst possible health state,” depending on the study and who was doing the calculating—and these are three very different things.

QALYs often rely on research that does not adequately account for the ways in which many people—especially, though not exclusively, those with rare conditions—may have medication responses that vary dramatically from the average . . .

Differences between patient subgroups may have a significant impact on the outcome of a CUA study. One study, which reviewed 200 of the 642 English-language CUAs in the Tufts Medical Registry, found that only 19 percent of these studies reported on any differences between subgroups.⁷⁰ Additionally, most studies only reported differences based on age.⁷¹ The authors hypothesized that failure to account for subgroup differences may lead to payers funding treatments that are of relatively low value or even harmful to some subgroups.⁷² Additionally, if payers only study subgroups for whom the treatment is of low value, they may not fund treatments that are of high value to some subgroups but of low value to others.

Different groups of patients, people with disabilities, or people with chronic illness may have dramatically different medication responses. QALYs often rely on research that does not adequately account for the ways in which many people—especially, though not exclusively, those with rare conditions—may have medication responses that vary dramatically from the average, either in terms of medication efficacy or side effects. This can create serious challenges under QALY-based systems, since a QALY calculation may result in a particular medication

being deemed cost-ineffective based on the average patient response, whereas for patients within a particular subgroup or who have atypical medication responses, it is the only medication that works or the only one that provides outcomes without terrible side effects.

Accounting for Clinical Knowledge Not Reflected in the Research Literature

For individuals with rare conditions or who come from groups underrepresented in research, like people with disabilities and people of color, the

inability of QALYs to account for information that primarily exists within clinical knowledge but has not yet made it into the research literature constitutes a serious problem. Many rare conditions do not have an adequate research literature to account for different subgroups or variation between patients in medication response. Since it can be difficult to study small populations, such knowledge may only exist on the part of the relatively small number of clinicians who specialize in treating such patients.

Chapter 2: Bioethics and Quality-Adjusted Life Years

There have been ethical objections to use of QALYs nearly since they were first invented. There are three primary ethical objections: (1) that disability may not actually reduce quality of life; (2) that QALYs discriminate against people with disabilities; (3) that QALYs fail to account for differences between what patients with the same condition value.

Does Disability Reduce Quality of Life?

Some stakeholders, but especially bioethicists and people with disabilities, have argued that

QALYs are built on a faulty premise: that life with a disability is inherently worse than life without a disability. As established in the section “Calculation of Quality- Adjusted Life Years,” QALYs work

by lowering the value of the life-extending properties of treatment (or the number of years the individuals being treated would normally have left to live) by the degree to which an illness or disability negatively impacts quality of life.⁷³ While QALYs are theoretically determining the “worth” of living in specific health states and not with specific disabilities (and from this,

the value of treatments that extend life or affect these health states), the reality is that people with specific disabilities have characteristics that match up with these health states. Being unable to walk, for example, is a core characteristic of paraplegia.

As described earlier in this report, QALYs typically evaluate the worth of a life with a disability based on the preferences of people from the general healthy population, most of whom do not have disabilities.⁷⁴ Disability rights advocates are rightly concerned that these preferences are not based on an accurate

Some stakeholders, but especially bioethicists and people with disabilities, have argued that QALYs are built on a faulty premise: that life with a disability is inherently worse than life without a disability.

understanding of what it is like to have a disability, but on stereotypes and a lack of understanding about disabilities. While some bioethicists believe that this can be mitigated by providing the general healthy population with

information about the conditions to help inform their responses, others see this as flawed, such as Dr. David Wasserman, bioethicist at NIH, who told NCD that there is a great deal of evidence that most of the general public and the medical profession in particular, overestimate the badness of being in various health conditions that are classified as disability.⁷⁵

Legal scholars Wendy Hensel and Leslie Wolf state that

quality of life considerations are not neutral, even when couched in mathematical terms, and are very likely to

be driven by prejudices and stereotypes concerning the desirability of life with disabilities. . . .

By favoring those with no functional impairments, the protocols implicitly endorse the belief that the lives of individuals without disabilities are more valuable than that of their unfortunate counterparts.⁷⁶

Although surveyors continue to rely on the healthy community's preferences for various health states, it is well known that this will skew the results of QALY analysis. The general population consistently rates life with a disability much more negatively than people with disabilities themselves do. In a study with more than 2,044 participants from the general US population, 47 percent of all participants rated

blindness as "the worst health condition that might befall them."⁷⁷ They ranked blindness as worse than AIDS, heart disease, the loss of a limb, and arthritis.⁷⁸ Bioethicist Sean

Sinclair, citing a UK study of more than 1,000 people, said that in this study 24 percent of those studied said needing to use a wheelchair for the rest of their life would be *worse than death*.⁷⁹

People with disabilities, however, consistently report that they get approximately the same degree of satisfaction from their lives as people without disabilities. One study reported that patients with "locked-in syndrome"—a disability

in which individuals are unable to move part or all of their bodies—self-report having a similar quality of life to people without disabilities.⁸⁰ An older 1979 study found that blind people, contrary to the beliefs

of the general population, were about as happy or slightly happier than people who could see.⁸¹ Gallaudet professors Dirksen Bauman and Joseph Murray have written that Deafness should be reframed from "hearing loss" to "Deaf Gain," in recognition of the ways in which Deaf people contribute to human diversity.⁸²

Does the Use of QALYs Discriminate Against People with Disabilities?

The use of QALYs may lead to the devaluing of treatments that extend the lives of people with disabilities. One of the earliest and most well-

known explanations of this problem was by Harris, who articulated his concerns in a 1987 journal article.⁸³ Harris argued that the use of QALYs would lead to a situation in which

funding treatments that extended the lives of people who could be restored to perfect health would be valued over treatments that extended the lives of people who could not be restored to

"By favoring those with no functional impairments, the protocols implicitly endorse the belief that the lives of individuals without disabilities are more valuable than that of their unfortunate counterparts."

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perfect health, such as people with disabilities and chronic illnesses.⁸⁴ Harris argued that it was morally unjust for QALYs to lead to the prioritization of the former over the latter.⁸⁵ Harris said we should adopt policies that “do not violate the individual’s entitlement to be treated as the equal of any other individual in the society.”⁸⁶

Disability rights advocates and people with disabilities oppose the use of QALYs for similar reasons.⁸⁷ Disability rights advocates are concerned that the widespread use of QALYs by health insurance companies and healthcare agencies will deny people with disabilities access to the care that they need.⁸⁸ Disability rights advocate Ari Ne’eman explained that such denials of care have in fact already happened to people with disabilities in countries that use QALYs more regularly. For example, as described in more detail in Chapter 3, the United Kingdom’s NICE determines which drugs Britain’s national health insurance program will cover by using QALYs. NICE recently denied coverage of three “groundbreaking” drugs for extremely rare and debilitating conditions.⁸⁹

Ne’eman’s article states:

All three drugs work by slowing irreversible organ damage and cell death. While they can and do improve current symptoms, their greatest promise is in halting or delaying disease progression. . . . Specialty drugs may still be able to add years to these patients’ lives, but NICE and other QALY-based systems discount the value of each of these years [because they are years lived with a disability.]⁹⁰

Proponents of QALYs argue that such a discount is irrelevant. They argue that QALYs are

not used to decide whether to treat individual patients,⁹¹ but, instead, to decide which treatments payers will fund.⁹² Bioethicist Greg Bognar states that if a treatment or drug is cost-effective, it will likely be covered. If it is covered, it will be offered to “all patients who need it, regardless of their other characteristics,” such as disability or race.⁹³ Some ethicists argue that in fact, if people with disabilities *are* assessed as having a low quality of life, a treatment that dramatically improved the types of quality of life measured by QALYS would probably be considered very cost-effective.⁹⁴

Additionally, they argue that the number of QALYs a person starts with before treatment does not matter. While people with disabilities seeking treatment for a disability will have lower “baseline” QALYs than a person without a disability, QALYs are primarily designed to determine the degree to which the treatment improves their health. Dr. Pearson provided an example during his interview which illustrates this point:

So, let’s say that you’re very sick and your quality of life is 0.3, and we have two treatments. We have a standard treatment, [which] improves the quality of life to 0.4 and we have one that raised quality of life . . . to 0.5. We’re trying to figure out which is most cost-effective. Now [next], I’ve got two other treatments for people that are going to start off at 0.8, which is pretty good. I’ve got the same two drug treatments—one makes you better by 0.1 and one makes you better by 0.2. The cost-effectiveness calculation is going to be exactly the same for those two comparisons among people that are very

sick, and the other among people that are pretty healthy. It's a comparison of how much better one is versus the other. . . . It doesn't matter where you start.

However, these arguments do not actually resolve the main concerns of QALY opponents such as Ne'eman—which is that use of QALYs may have the effect of devaluing treatments primarily designed for a population of people with a chronic illness or disability, in practice. If the primary purpose of QALYs is to allow decision makers to determine how best to spend money on health care, which proponents of QALYs do not dispute, then almost necessarily these decision makers are comparing unlike treatments and deciding which of these to fund. As

established in Example 1 about Connie and Bill, patients with chronic illnesses and disabilities who retain their disability

after treatment do not just start with fewer QALYs than people who can be restored to perfect health—they also *gain* fewer QALYs from treatment than people who can be restored to perfect health. As noted in the section “Methodological Flaws of Quality- Adjusted Life Years” and earlier in this section, there are likely many classes of both treatments, drugs, and the patients they serve where this is the case. Use of QALYs will therefore prioritize treatments like the one for Bill rather than treatments like the one for Connie, even if what is measured is how many QALYs both would gain from treatment.

Health insurers are also not merely choosing between treatments within conditions, although some proponents of QALYs claim as much. Researchers and health economists have

repeatedly stated that the primary purpose of QALYs is to allow decision makers to compare the cost-effectiveness of treatments for unrelated conditions.⁹⁵

Further, use of QALYs would not be necessary if health insurers were comparing the cost-per-QALY or QALYs gained from only related treatments. Chapter 5, “Alternatives to the Use of QALYs,” describes other ways that payers may compare the cost-effectiveness of different treatments for the same condition without the use of QALYs. It is unlikely, after the passage of the Affordable Care Act, that payers in the United States would refuse to cover an entire class of patients, and QALYs would not act as justification for doing so. However, even if a payer treats all classes of patients, the quality of some classes

of patients' care may be worse, or their options more limited, because some of the potential treatments available

to them were not deemed cost-effective and therefore not covered by their insurance due to the impact of their disability on QALY calculations.

Harris had an additional objection that is also of significance. In the real world, payers rarely face a choice between treating two disabilities of equal severity. Instead, payers more often face a choice akin to providing a little bit of quality of life to many people versus saving one person's life. For example, a health insurance provider with a limited amount of money may have to choose between funding hip replacement surgery for many people, and funding a high-cost treatment that saves the lives of only a few people with a rare disease. QALYs do not distinguish between the two types of treatment.⁹⁶ If funding hip replacement surgery

“That’s like saying that drugs for cystic fibrosis are also unavailable to patients without cystic fibrosis.”

for a hundred people obtains more QALYs than funding the high-cost treatment, then funding the hip replacement surgery will more than likely be given higher priority, even if the high-cost treatment saves lives. As Harris points out, this is quite inconsistent with the moral intuitions of many people.⁹⁷

More significant ethical problems exist when the only class of drugs known to be effective for a certain group of patients with disabilities is not

covered because the drugs are not considered cost-effective.⁹⁸ In that situation, it does not matter that QALYs are theoretically meant to be used to evaluate treatments rather than patients. As Ne'eman wryly stated: "That's like saying that drugs for cystic fibrosis are also unavailable to patients without cystic fibrosis." Chapter 3 provides specific examples of situations in which just such a problem has happened in other countries.

Chapter 3: Utilization of QALYs in the United States

Introduction

QALYs have a complicated history of use in the United States. Although QALYs are frequently used in research, their use to determine benefits and coverage has historically been more limited compared to their use for this purpose in other countries. There are likely multiple reasons for this; some health economists attribute it to the United States' cultural aversion to metrics that may discriminate, or the United States' multi-tier, complex healthcare system.⁹⁹ To understand this complex usage history, NCD undertook a comprehensive review of how QALYs are used in the United States.

Use of QALYs by the US Federal and State Governments

There is no one, singular policy on the use of QALYs across the entirety of the US government. Each federal agency has a distinct and separate policy, although the overall use of QALYs has followed a pattern over time. QALYs grew in popularity as a measure of cost-effectiveness during the 1990s to

2000s, declined in popularity due to failed implementations of the metric during that time and the passage of the Affordable Care Act, and have recently increased in prominence and popularity due to concerns about rising healthcare costs in the United States.

One of the most prominent attempts to utilize QALYs in a state-run health insurance program was found to violate the Americans with Disabilities Act (ADA). Starting in 1989

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and continuing into the early 1990s, the state of Oregon attempted to reform its Medicaid program by ranking treatments in terms of their cost-effectiveness.¹⁰⁰ Oregon

created a list of more than 700 paired treatments and diagnoses (an example of a paired treatment and diagnosis on the first list was "Diagnosis: mental disorders with no effective treatment; Treatment: evaluation") and decided it would cover the 587 most cost-effective items on the list.¹⁰¹ Oregon ranked these pairs according to 13 criteria.¹⁰² Oregon used QALYs in order to measure some of these criteria, particularly quality of life and life expectancy.¹⁰³

The use of QALYs produced counterintuitive results: capping teeth was ranked above

appendectomy as it produced more QALYs for more people in the aggregate, even though an appendectomy saves a life.¹⁰⁴ The Bush administration ultimately rejected Oregon's Medicaid plan, as it was found to violate the Americans with Disabilities Act.¹⁰⁵ A Bush administration official stated in a letter to the editor sent to the *New York Times* that the plan was rejected because it "in substantial part values the life of a person with a disability less than the life of a person without a disability."¹⁰⁶ Oregon's Medicaid program has continued to ration care according to cost-effectiveness, however.¹⁰⁷

From the 1990s to the late 2000s, different Federal Government agencies considered how (and where) the Federal Government should utilize cost-effectiveness research. Each of these agencies came to different conclusions about use of QALYs. For instance, in 2007 the Congressional Budget Office (CBO) expressed concerns about QALYs in a paper titled *Research on the Comparative Effectiveness of Medical Treatments: Issues and Options for an Expanded Federal Role*.¹⁰⁸ In the paper, the CBO argues that the United States should take more of a role with respect to promoting the use of comparative effectiveness research.¹⁰⁹ One of the ways the

A Bush administration official stated in a letter to the editor sent to the New York Times that the plan was rejected because it "in substantial part values the life of a person with a disability less than the life of a person without a disability."

The trend toward QALY usage changed with the passage of the Affordable Care Act in 2010. Certain federal agencies, particularly health-related agencies, were prohibited or severely limited in how they could utilize QALYs by the Affordable Care Act.

CBO proposes doing this is by creating a new federal entity that commissions, performs, and evaluates comparative effectiveness research and how it relates to policy.¹¹⁰ The paper evaluates

cost-effectiveness in this context. It notes that the use of "common metrics like QALYs" may "raise concerns among patients" and other stakeholders.¹¹¹

In 2006 the Department of Health and Human Services

evaluated the cost-effectiveness of one of its population-wide vaccination programs using "years of healthy life saved," a direct reference to the use of QALYs.¹¹² The US Public Health Service's "Healthy People Initiative," which measured progress toward US public health goals, in 2006 used QALYs "as one of its key metrics."¹¹³ Throughout the late 1990s and the early and mid-2000s the US Food and Drug

Administration (FDA) utilized QALYs as part of its agency rulemakings.¹¹⁴

The trend toward QALY usage changed with the passage of the Affordable Care Act in 2010. Certain federal agencies, particularly health-related agencies, were prohibited or severely limited in how

they could utilize QALYs by the Affordable Care Act. 42 U.S. Code § 1320e-1(e), which came from the Affordable Care Act, prohibits the Patient Centered Outcomes Research Institute (PCORI)

from using QALYs or any other similar measure that “discounts the value of a life because of an individual’s disability,” as a “threshold” for determining what type of health care is cost-effective.¹¹⁵ It also prohibits PCORI from using QALYs when developing healthcare coverage, incentives, or reimbursement programs.¹¹⁶

Medicare is similarly prohibited from utilizing “cost-effectiveness research” (a much more general term that applies to more than just QALYs) in a manner that treats “extending the life” of an elderly, ill, or disabled person as of less value than “extending the life” of someone who is none of the above.¹¹⁷

Medicare *can* use cost-effectiveness research if it is instead used for

“determining coverage, reimbursement, or incentive programs under subchapter XVIII

based upon a comparison of the difference in the effectiveness of alternative treatments in extending an individual’s life due to the individual’s age, disability, or terminal illness.”¹¹⁸

This may mean that Medicare can use cost-effectiveness research to compare related treatments to one another, such as two different treatments that extend the life of someone with cystic fibrosis, and consider how disability impacts the degree to which these treatments extend life. However, the exact meaning of the phrase is ambiguous.¹¹⁹

The use of QALYs among federal agencies has increased in recent years. Dr. David Wasserman, at the National Institute of Health’s Department of Bioethics, said that “use of QALYS has modestly increased in the face of opposition. It is used by at least one US agency . . . Some sort of cost-effectiveness analysis is commended

to various agencies. I could say that there is a general trend toward quantifying outcomes. There’s a related overlapping trend to use patient reported outcome measures for quality of care assessments, which may appeal to a broader constituency and patient advocacy groups.”¹²⁰

The Department of Veterans Affairs (VA)’s PBM Services office utilizes the HTA reports produced by ICER (described in the Introduction and Chapter 1) to aid the development of its drug formularies, which generally means the lists of drugs that a health insurer will cover, although sometimes a health insurer will cover a drug not listed on its formulary.¹²¹ ICER’s reports, as stated, utilize QALYs. The VA’s formulary

development process is well-developed, extensive, and utilizes many forms of data other than ICER’s reports.¹²²

The VA does not utilize a cost-effectiveness threshold.¹²³

Use of QALYs by Private Health Insurers

Limited information is publicly available on the degree to which private insurance companies utilize QALYs to make benefits and coverage decisions. According to most scholarly sources, QALYs are rarely explicitly used by health insurers in the United States. Louis P. Garrison reported in his 2016 article that US private payers, with a few limited exceptions, rarely explicitly used cost-utility analyses (CUAs), the cost-effectiveness studies that rely on QALYs, in their benefits and reimbursement decisions.¹²⁴ He stated that it was a “puzzle” that the United States had so many competent health economists who made so many CUAs, but that US private and public payers

QALYs are rarely explicitly used by health insurers in the United States.

rarely made direct use of their material.¹²⁵ Health economist Peter Neumann has said in multiple¹²⁶ articles¹²⁷ that QALYs are rarely used explicitly for benefits and coverage decisions in the United States.

For many health insurers, use of QALYs or QALY-based valuations may instead be implicit, and part of an internal decision-making process over which there is little transparency or oversight. Eleanor Perfetto, Executive Vice President of Strategic Initiatives for the National Health Council, an organization which developed a Patient-Centered Value Model Rubric that is used to evaluate the patient-centeredness of value frameworks,¹²⁸ said at the September 2018 NCD stakeholder convening:

There's not much documentation . . . They may or may not have used QALYs. We don't know. But if they did . . . [use] them in their decision making, it probably isn't well documented . . . And even if it is, it's not public information. . . . or [they've] been used in terms of publications that might come out that people might put in journal articles, [such cost-effectiveness studies by researchers], for others [such as health insurers] to use or to consider in their decision making.

One important interview supported a similar conclusion. In Spring 2016, the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) produced a Special Task Force Report on US value frameworks. As part

of its research, ISPOR interviewed members of key stakeholder groups, including Brian Solow and Edmund J. Pezalla, who are PBM representatives.¹²⁹ Solow and Pezalla were asked questions on the extent to which insurance agencies utilized cost-effectiveness research and value frameworks in decision making. Solow reported that "maybe they do," but that with the exception of a few small plans, "nobody has a clinical policy that says we're constructing this on cost-effectiveness grounds."¹³⁰ This appears to mean that, while cost-effectiveness is used, there is no explicit written policy that would require insurers to make decisions based on cost-effectiveness.

Solow and Pezalla were also asked to what extent payers used the value frameworks ISPOR investigated in its report. Solow and Pezalla reported that "everybody" read ICER's reports, which rely on QALYs.¹³¹ However,

Solow and Pezalla also reported that payers rarely followed the recommendations made in ICER's reports "to the letter."¹³² According to the two managers, many plans do not rely on QALY-related aspects of these value frameworks, and instead attempt to do "the economic calculation without the QALY," while taking the clinical and economic evidence ICER used to generate QALYs or the cost-per-QALY into account.¹³³

Several of the individuals that NCD researchers interviewed did not agree with these statements. These individuals felt that private health insurers' interest in QALYs had been steadily increasing over the last few years.

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Sara van Geertruyden of the Partnership to Improve Patient Care said that use of QALYs was “increasing” and that CVS Caremark’s announcement in 2018 of their intent to base their benefits and coverage decisions on ICER’s QALY-based reports¹³⁴ indicates that “private plans and pharmacy benefit managers are referencing QALY-based reports [such as ICER’s] and using them to inform coverage and formularies.” Ne’eman similarly stated¹³⁵ that, while QALYs have been used in academic contexts for some time, that they have had “increased utilization” in recent years in the benefits and reimbursement context by PBMs, citing the recent proposal from CVS Caremark to adopt a QALY threshold.

Van Geertruyden referenced a specific situation in which consideration of QALYs by health insurers had a specific impact on a population of patients in the United States. The incident involved two anti-cholesterol drugs, Praluent and Repatha, which target a protein known as PCSK9.¹³⁶ As van Geertruyden explained, “Certain patients with genetic, familial high cholesterol (FH) and some other patients don’t respond well to statins [commonplace drugs that reduce high cholesterol]. PCSK9s are designed for this population.”¹³⁷

Unfortunately, the first clinical study available on a PCSK9 (Repatha) was of a general population who were at relatively low risk for heart attack and stroke, rather than the patients with high cholesterol that the drug was actually intended to treat.¹³⁸ Consequently, some of the benefits of the drug (such as prevention of deaths) appeared lower than they actually were.¹³⁹ An initially high cost-per-QALY for these two medications was reported by ICER and, partially as a result of that report, according to

van Geertruyden, as well as the higher initial cost of the drug, countless patients who did need the drug were denied it.¹⁴⁰

The evidence presented neither indicates that QALYs are a controlling variable for all health insurance decisions in the US nor that QALYs are not used by health insurers at all. While few health insurance agencies explicitly mention cost-effectiveness as the basis for their decisions, QALYs and the cost-effectiveness research they support are most likely important evidence that supports and guides, rather than mandates, various courses of action that private health insurers could take.

Ethical Concerns with Respect to the Use of QALYs in the United Kingdom and Their Relationship to Concerns in the United States

The concerns of disability rights advocates, bioethicists, and patient rights groups in the United States who oppose widespread use of QALYs are informed by their use in countries where QALYs play a much more significant role in healthcare decision making. QALYs are a key metric used by the United Kingdom’s NICE.¹⁴¹ The primary purpose of NICE is to decide which drugs and treatments will be funded by Britain and Wales’ national healthcare system, the NHS.¹⁴² To do this, NICE analyzes how cost-effective each new drug or treatment is by calculating the treatment’s cost per quality-adjusted life year.¹⁴³ NICE publicly publishes its analyses of each new drug or treatment, which it refers to as “health technology appraisals” or “guidance.”¹⁴⁴

NICE’s reports are known to reduce patients’ access to care. This is particularly likely to happen to patients who have a complex condition which

may require intensive, expensive treatment in order to manage it—which describes many people with disabilities.¹⁴⁵ For example, NHS patients lack unrestricted access to most cancer drugs. According to a 2018 Avalere Health study of over 329 HTAs of cancer drugs created by governmental agencies between 2013 and 2017, NICE recommended access restrictions for nearly 70 percent of the cancer drugs it assessed, and it rejected 22 percent of the cancer drugs.¹⁴⁶ By contrast, in the United States, cancer patients gain access to cutting-edge medications earlier and are diagnosed earlier¹⁴⁷ than in the United Kingdom. For some cancers (such as lung cancer) US patients have a higher survival rate than UK patients, which is related to their quicker access to diagnosis and medication.¹⁴⁸

Alzheimer’s Disease

One prominent example of how NICE’s QALY-reliant reports can have a negative impact on patients was its 2005 rejection of the drugs donepezil, galantamine, rivastigmine and memantine for use by patients with mild to moderate Alzheimer’s Disease.¹⁴⁹ Alzheimer’s Disease is a progressive neurological disease that, over time, reduces and eventually eliminates the affected person’s ability to learn and remember new information.¹⁵⁰ The four drugs are standard treatments for Alzheimer’s Disease, and mainly maintain rather than improve the affected person’s functioning.¹⁵¹ According to patients with the disease and their families, they significantly benefit from

maintaining their functioning at earlier stages of the disease.¹⁵²

NICE’s draft recommendations nonetheless found that the drugs were not cost-effective despite evidence of this benefit to patients.¹⁵³ Notably, the drug donepezil (Aricept) only cost 2.50 pounds per day per patient in 2007, only 2 years after the draft guidance was released, which at the time was around the price of a cup of coffee.¹⁵⁴

NICE’s recommendations were widely criticized by patients and other prominent stakeholders in the United Kingdom.¹⁵⁵ Several

criticisms focused on the validity of QALY calculations used by NICE. The Royal College of Psychiatrists, for example, argued that it made no clinical sense to deny patients with mild and moderate forms of the disease access to the medications, as these would be the very patients who would

obtain a greater benefit from retaining a higher level of functioning for longer.¹⁵⁶

Some researchers and doctors argued that using a quality-of-life focused measure was improper given that it is difficult to estimate health-related quality of life in patients with a progressive neurological disorder.¹⁵⁷ It is difficult to translate the small but important cognitive or behavioral gains from these drugs into evidence of clinical efficacy in controlled conditions.¹⁵⁸ Most evaluations of the quality of life of patients with Alzheimer’s Disease were based on the responses of doctors or caregivers, and it was

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known that the use of different proxies produced different results, bringing the validity of the utility values into question. Additionally, some individuals have argued that NICE's recommendations were based on limited empirical data that, where it did exist, was entirely invalid when applied to some categories of patients. NICE's 2005 recommendations were based primarily on a US study of Alzheimer's Disease patients who took a specific cognitive functioning test known as the Mini Mental State Exam (MMSE), which the Royal College of Psychiatrists in the United Kingdom argued was highly influenced by age, sex, and English proficiency and was invalid for patients with intellectual and developmental disabilities.¹⁵⁹

These heavy criticisms prompted NICE to revise its guidelines in 2006, which still restricted access,¹⁶⁰ and led to significant legal challenges by trade associations and the pharmaceutical industry.¹⁶¹ These efforts failed,¹⁶² and patients with mild Alzheimer's Disease in the United Kingdom were unable to obtain the drugs until 2010, when NICE again changed its guidelines, likely due to a committed campaign by patients and patient rights organizations.¹⁶³ Currently, NICE recommends the use of the first three drugs for all patients and the last drug for patients with severe Alzheimer's Disease.¹⁶⁴

To a certain extent, the limitations NICE imposes on patient access to care in England and

Wales are mainly due to the United Kingdom's national healthcare system. The NHS has a limited budget and yet must provide care to all citizens. The issue of how to allocate scarce funds is therefore particularly pressing. A UK reporter argued that the United Kingdom has no choice but to limit patient access to high-cost treatments, even if it means utilizing metrics such as QALYs, because paying for high-cost drugs depletes the NHS's funds and therefore its capacity to serve many more people than the few who benefit from a high-cost treatment.¹⁶⁵ However, similar problems exist in the US's

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national healthcare programs, which must provide a basic level of care to everyone who is eligible. While this type of rationing may be inevitable in healthcare, it nonetheless poses an existential threat to many people with disabilities. Crucially, there may be alternatives to the use of the quality-adjusted life year. For more

information on the alternatives that have been proposed, see Chapter 5, "Alternatives to the Use of QALYs."

Cystic Fibrosis

NICE's treatment of the cystic fibrosis drug Orkambi (lumacaftor/ivacaftor) illustrates the risks QALYs pose to people with rare and complex conditions even when the cost-effectiveness assessment does not assign patients a markedly reduced health utility value. Cystic fibrosis is a genetic disease which causes thickened mucus

secretions to progressively block the lungs and digestive system.¹⁶⁶ Eventually, most people with CF will die from respiratory failure.¹⁶⁷ In 2017, the median age of death for patients with CF in the UK was 31 years.¹⁶⁸

Until recently, only treatments for the symptoms of CF existed. Nebulized medications such as Pulmozyme and hypertonic saline thin mucus so it is easier to clear, but do not correct the defect leading to the production of thickened mucus.¹⁶⁹ Orkambi, manufactured by Vertex Pharmaceuticals, is a member of a new class of drugs known as CFTR modulators.¹⁷⁰ These drugs partially restore correct production and function of the protein that is defective in cystic fibrosis.¹⁷¹ Each CFTR modulator is only clinically appropriate for a subset of CF patients with specific mutations.¹⁷²

In July 2015, the FDA approved Orkambi for patients 12 years and older with homozygous F508del mutations.¹⁷³ About half of CF patients in both the United States and the United Kingdom have this genotype. NICE issued an initial rejection in mid-2016, estimating the drug's incremental cost-effectiveness ratio to be between £218,248 to £349,337 per QALY (approximately \$280,000 to \$460,000 per year; the lower value relies on the assumption that after 10 years, prices would be reduced by the introduction of a generic).¹⁷⁴ NICE's officially recommended cost-effectiveness threshold window is far below this, ranging from £20,000 to £30,000 per QALY.¹⁷⁵

The detailed justification of NICE's cost-effectiveness assessment illustrates the problems with attempting to capture treatment benefits perceived by people with disabilities using general population measures. Though many adults with CF have significant functional

limitations and may spend weeks per year in the hospital or on home IV treatments, patients often give high ratings on general quality of life (QoL) scales.¹⁷⁶ Patients in Vertex's study gave baseline health-related QoL ratings on NICE's preferred instrument that corresponded to a median health utility value of 1, equivalent to the healthy, nondisabled population.¹⁷⁷ This left no room for subjective improvement in quality of life. The NICE appraisal states that "both the clinical and patient expert explained [to the committee] that people with cystic fibrosis may perceive their health-related quality of life to be equivalent to that of people without cystic fibrosis because they have never known any other health state."¹⁷⁸ However, the committee "understood from the clinical experts that they considered that the 5 dimensions of the EQ-5D questionnaire generally captured most of the important effects of cystic fibrosis" and deemed there to be insufficient evidence that the general population measure was inappropriate.¹⁷⁹ As a result, the estimated cost-per-QALY for Orkambi could only incorporate its predicted longevity benefit.

In the United States, ICER has also used the QALY to evaluate Orkambi's cost-effectiveness.¹⁸⁰ ICER chose to assign health utility values based on a measure of patients' lung function.¹⁸¹ A CF patient's health utility value could be at minimum 0.625 and at maximum 0.92.¹⁸² This meant that the expected reduction in rate of disease progression could be reflected in increased amounts of time at higher utility values. However, this degree of discounting meant that ICER's assessment resulted in an incremental cost-effectiveness ratio of \$890,700 per QALY,¹⁸³ much higher than NICE's estimate (and providing justification for potential denial of coverage by payers).

In both evaluations, patients are disadvantaged by the forced tradeoff between increased length and quality of life. Additionally, the discrepancy in methods and assessed treatment value make the metric's claimed objectivity seriously questionable.

Three years after NICE's initial rejection, CF patients still do not have access to Orkambi on the English NHS. In the summer of 2018, NHS England offered to cover all of Vertex's existing and future therapies at a 90 percent reduction from the list price.¹⁸⁴ This would amount to less than £10,000 per patient per year.¹⁸⁵ This cost is less than that of Pulmozyme, a symptomatic treatment first approved by the US FDA in 1993.¹⁸⁶ Vertex has refused this offer, stating that it would set a precedent for price negotiations in other countries that would make funding further research and development impossible.¹⁸⁷

Use of Similar Models in United States National Health Insurance Programs

Disability and patient rights advocates have expressed concerns that, as the United States increasingly attempts to find ways to save money in healthcare contexts, it will look towards modeling its own national health insurance programs after those in the United Kingdom

and other countries that use QALYs. Some US government agencies are already investigating the prospect of doing so. The Centers for Medicare and Medicaid recently published an Advance Notice of Proposed Rulemaking (ANPRM) which proposes an International Pricing Index (IPI).¹⁸⁸ The IPI would base the prices of certain drugs covered under Medicare Part B on reference prices from 16 other countries. Many of these countries—for instance, the United Kingdom, Ireland,¹⁸⁹ and Canada¹⁹⁰—use QALYs to make benefits and coverage decisions and limit their healthcare costs. At the state level, the Drug Utilization

Review board in New York voted unanimously in April 2018 to recommend that state Medicaid payments for Orkambi be reduced by 70 percent in order to meet ICER's recommended

maximum threshold of \$150,000 per QALY. Drug manufacturers are unlikely to accept such extreme price reduction demands, posing a threat to treatment access for patients in states choosing to enforce cost-effectiveness thresholds.

The failure of Oregon's initial waiver is instructive. While some consideration of cost-effectiveness is reasonable in national health insurance programs, strict prioritization that is overly reliant on QALYs, similar to the kind utilized in the United Kingdom, is contrary to US civil rights law and disability policy.

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Chapter 4: Case Study: CVS Caremark

Introduction

NCD's case study for this report investigates one particular upcoming use of the quality-adjusted life year in the United States: the PBM CVS Caremark's recent decision, in August 2018, to allow self-insured employers to exclude drugs from their formularies that were found to not be cost-effective, based on the cost exceeding a threshold of \$100,000 per QALY.¹⁹¹ CVS Caremark's decision is controversial. A wide variety of stakeholders have spoken on how CVS Caremark relates to the viability of QALYs as a means to cut healthcare costs and aid healthcare coverage decisions in the United States. While some stakeholders lauded the decision as a victory that would drive down costs for consumers, others were concerned that CVS Caremark's use of QALYs would lead to blanket, one-size-fits-all coverage decisions that would prevent people with disabilities from accessing the medications and treatments that they need.

Background

CVS Caremark is a type of company known as a pharmacy benefit manager, or PBM. PBMs contract with health insurers and employer

sponsors of health insurance plans and act as administrators of their prescription drug benefits.¹⁹² Their clients are diverse, and can be private health insurance companies, employer sponsors of employee health insurance plans, and state Medicare and Medicaid agencies, among others.¹⁹³ While PBMs began largely as "middlemen" who processed health insurance claims, they now have many other important roles in the health insurance industry.¹⁹⁴ Modern-day PBMs can: (1) help determine which drugs will be covered by aiding in the development of drug formularies; (2) make reimbursement decisions, deciding how much pharmacies in their client's network will be reimbursed for their services; and (3) operate pharmacies themselves.¹⁹⁵

PBMs, given that they manage the prescription drug benefits of more than 266 million Americans according to the Pharmaceutical Care Management Association,¹⁹⁶ have significant influence over what drugs are and are not covered by health insurance. According to Ne'eman, PBMs are, from the insured person's perspective, "payers themselves."¹⁹⁷ CVS Caremark is a particularly large PBM. CVS Caremark, along with two other PBMs, Express Scripts and OptumRx, administer 70 percent of

all PBM-managed prescription drug claims in the United States.¹⁹⁸ Any action CVS Caremark takes, therefore, has an impact on the lives of millions of Americans.

CVS Caremark's Decision

In August 2018, CVS Caremark released a white paper titled, *Current and New Approaches to Making Drugs More Affordable*. The white paper described the steps that CVS Caremark intends to take to reduce the cost of prescription drugs in the United States.¹⁹⁹

One of the steps CVS Caremark described in its white paper is "Reducing Launch Price Using Comparative

Effectiveness." In

the white paper, CVS Caremark stated that parts of Europe have a loose cost-effectiveness threshold of \$50,000

per QALY, which in CVS Caremark's view

encouraged drug manufacturers in Europe to launch new

prescription drugs at lower prices in order to meet this threshold.²⁰⁰ CVS Caremark stated that the US "does not have any such programs," and that therefore the launch prices of new prescription drugs in the United States continues to rise.²⁰¹

CVS Caremark then explained that it was launching a new program, which would allow some of the PBM's clients to exclude from their drug formularies any drug with a launch price greater than \$100,000 per QALY.²⁰² CVS Caremark would use the HTAs produced by ICER to determine whether a drug's launch cost-per-QALY

fell below or at the threshold.²⁰³ CVS Caremark's policy is only available to self-funded insurance plan sponsors, who are mostly employers.²⁰⁴ CVS Caremark's policy does not affect "breakthrough therapies," which are medications that the Food and Drug Administration deems more effective at treating a "serious or life-threatening" condition than existing therapies.²⁰⁵ CVS Caremark's theory was that if enough PBM clients agree to exclude drugs from their formularies in this manner, drug manufacturers will be forced to lower the launch prices of their drugs.²⁰⁶

Responses to the CVS Caremark Decision

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CVS Caremark's decision attracted controversy as soon as it was published, with both positive and negative responses written in response to CVS Caremark's announcement.

Positive responses emphasized the significant role that

drug manufacturers play in driving up the price of prescription drugs, and saw CVS Caremark's policy as a "bold move" to curtail expanding launch prices.²⁰⁷ Max Nisen, a *Bloomberg Opinion* columnist, stated that CVS Caremark's policy was a positive change but that it "did not go far enough," suggesting that CVS Caremark should also exclude "breakthrough therapies" as they were becoming more commonplace and were often highly expensive.²⁰⁸ The online magazine *Vox*, summarizing the statements of Dr. Wallid Gellad, stated that "Stricter formulary designs are one of the few direct tools that might be

able to influence drug manufacturers' behavior," and Gellad said that "something like this is the inevitable future."²⁰⁹ However, Gellad criticized CVS Caremark's exclusive use of ICER's cost-effectiveness analyses, stating that "the idea that we base something solely on a cut point determined by one cost effectiveness analysis from ICER is a big step to take."²¹⁰ Gellad, like Nisen, also wondered if the new program would actually impact that many drugs, given that it would exclude high-cost "breakthrough" drugs.²¹¹

Negative responses emphasized the arbitrary nature of the \$100,000 cost-per-QALY threshold, the inability of QALYs and other kinds of cost-effectiveness to fully gauge a medication's worth to patients, and the danger that the use of QALYs will greatly reduce access to care. Robert W. Dubois, of the National Pharmaceutical Council, stated that evaluating all medications for all conditions using a single \$100,000-per-QALY cutoff threshold was "inappropriately blunt" and arbitrary. Dubois noted that most other entities that use cost-effectiveness, including ICER itself, either use variable thresholds (such as between \$100,000 to \$150,000 per QALY) or do not use their threshold as an absolute cut-off point.²¹² He stated that a singular threshold did not account for significant differences between how different patients with the same condition can respond to a medication.²¹³ Two subgroups of patients with the same condition could receive a different number of QALYs, and thereby a different cost per QALY²¹⁴ would be calculated for the drug. Dubois also said that CVS Caremark's plan failed

to account for societal benefits of a drug, such as reduced caregiver burden or increased productivity.²¹⁵

Patient rights organizations shared Dubois' concerns and additionally criticized CVS Caremark's proposed use of the quality-adjusted life year itself. Tony Coelho of the Partnership to Improve Patient Care (PIPC) argued that CVS Caremark's new policy, by relying on QALYs, would discriminate against people with disabilities and elderly people in the ways described in Chapter 2, "Bioethics and the Quality-Adjusted Life Year,"²¹⁶ in that QALYs will undervalue treatments for people with chronic conditions and disabilities who can never be returned to "perfect health," as defined by researchers using QALYs. Ninety patient and disability rights organizations signed onto a September 2018

Ninety patient and disability rights organizations signed onto a September 2018 letter to CVS's CEO, Larry Merlo, which opposed the policy.

letter to CVS's CEO, Larry Merlo, which opposed the policy.²¹⁷ Disability rights advocates raised similar concerns, and highlighted the particularly negative impact of such a policy on people with rare diseases and conditions.²¹⁸

Some news outlets primarily commented on the relationship between CVS Caremark's new policy, the Institute for Clinical and Economic Review, and QALYs. Economics magazine *Forbes*, for instance, commented that ICER's methodology was very similar to the methodology used by the United Kingdom's NICE agency, and titled its article, "Will CVS Caremark Make ICER the American NICE?"²¹⁹ ICER has defended its use of QALYs in response to the widespread criticisms of the metric by patients

and disability rights groups. An ICER representative stated the following:

QALY is recognized as the gold standard for measuring how much a treatment improves patient lives, and it effectively rewards innovative medicines that significantly improve the lives of patients most in need. Patient populations that start off with a lower quality of life—whether because of a serious chronic illness or disability—actually represent the greatest opportunity for treatments to achieve a significant improvement in QALYs.²²⁰

CVS Caremark’s Response to Criticisms and Stakeholder Concerns

CVS Caremark’s initial response to the criticisms has been limited. In a *HealthAffairs* blog article responding to Dubois, CVS representatives Troyen Brennan and Surya Singh explained that the cost per QALY is determined by both the medication’s impact on “quality of life” (as measured by QALYs) and the price the manufacturers set for the drug.²²¹ Given this, a manufacturer could lower the drug’s cost-per-QALY by setting a lower launch price for the drug.²²² The article did not address concerns that QALYs inherently undervalue certain categories of patients, and describes QALYs as a “quantitative method” that “help[s] stakeholders compare the costs and effectiveness of medications.”²²³ They also do not address Dubois’ concern that a singular cost-per-QALY threshold does not account well for situations in which

different groups of patients respond differently to a medication and thereby generate different cost-per-QALY estimates for the same drug.²²⁴

An article by *STAT News* in September 2018 reports that CVS Caremark is engaged in discussions with representatives of some of the 90 groups that signed PIPC’s September 12th letter.²²⁵ Troyen Brennan, CVS’s Executive Vice President said, “It behooves us to spend some time to understand the concerns of the disability community and, if necessary, modify the measures so the process treats every life as being of equal value. We’ll go with the program we have now, but we’re looking for ways that we might modify it down the line.”²²⁶ As of the time the article was written, CVS Caremark’s policy was still set to begin in 2019.²²⁷

Conclusion

As of February 2019, there was no news available that indicates the impact of CVS Health’s implementation of its new policy. Its ultimate effect on patient access to prescription medications is therefore unknown. The discussion surrounding CVS Caremark’s new policy, however, brought the QALY into the public eye. CVS Caremark’s status as one of the largest pharmacy benefit managers in the United States meant that its change in policy could have an impact on millions of Americans, particularly Americans with disabilities. Central to the debates about CVS Caremark’s policy was its use of QALYs, and whether or not it can be used as a tool to control rising prescription drug costs without harming patients with chronic illnesses and people with disabilities. Some individuals

“Will CVS Caremark Make ICER the American NICE?”

lauded CVS Caremark's attempt to bring down prescription drug costs, while others raised reasonable concerns about CVS Caremark's use of both a bright-line cost-effectiveness threshold and the flawed but ubiquitous QALY. NCD presents this case study as an overview

of the arguments for and against use of QALYs in benefits and coverage decisions, and recommends that the Department of Health and Human Services carefully consider all of the issues and avoid the use of QALYs or any similar metric in its own health programs.

Chapter 5: Alternatives to the Use of QALYs

Various alternatives to the use of quality-adjusted life years have been proposed. These alternatives differ from one another in a variety of ways, including: (1) whether or not the alternative attempts to serve all of the same functions as QALYs; (2) whether the alternative uses the same means of assessing which treatments are most “valuable” as conventional QALYs, or whether it uses a different means of assessing the “value” of a treatment; and (3) whether the alternative has actually been used in practice, or whether it is only theoretical.

Equal Value of Life Years Gained (evLYG) Supplementary Measure

In response to criticism from disability rights activists regarding the QALY, in December 2018, ICER announced their intent to use a supplementary measure in addition to the QALY, entitled the equal value of life years gained (evLYG). The evLYG is intended to act as a supplement, rather than a replacement, for the QALY. It offers an additional unweighted measure of years of life extended utilizing particular

treatments (without the reduction in value of a year of life extended created by the use of a health utility or disability weight), intended to allow an observer or payer to see if there is a significant discrepancy between the QALY and evLYG outcome. Early use of the evLYG indicates

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that there are such discrepancies. For example, in ICER’s analysis of Spinraza, a new breakthrough therapy for Spinal Muscular Atrophy with significant life-extension potential, ICER concluded that utilizing a \$100,000

to \$150,000 per Quality-Adjusted Life Year (QALY) threshold, Spinraza’s maximum permissible reimbursement level for people with presymptomatic SMA would be \$72,000 to \$130,000 for the first year of treatment and between \$36,000 to \$65,000 for each successive year. Utilizing the evLYG at the same monetary threshold, the maximum permissible reimbursable price would be between \$83,000 to \$145,000 during the initial year and \$41,000 to \$72,000 for each successive year. Both are significantly below Spinraza’s cost of \$750,000 for the initial year and \$375,000 per year thereafter, suggesting that Spinraza would not be covered

under QALY systems or systems that utilized the QALY and the evLYG together. (In the United Kingdom, Spinraza is not covered due to the QALY analysis conducted of the drug by NICE.)

There are other challenges to the evLYG that indicate that it is not a suitable alternative to the QALY. First, as evidenced by the assessment of Spinraza, denial of coverage is possible under the QALY/evLYG system, even where a drug would provide significant clinical benefit, including life extension. Second, the QALY/evLYG system still relies on health utility weights to measure quality of life improvements, despite the fact that such measures are typically derived from survey data and do not account for the complexity of the preferences and experiences of people with disabilities. Third, the QALY/evLYG system affords no opportunity to account for clinical knowledge not reflected in the research literature, a significant concern articulated in Chapter 1. Finally, even within the narrow emphasis on life extension, ICER provides no guidance to payers as to which reimbursement level to prioritize—the one derived from the QALY or the one derived from the evLYG.

Not Using QALYs When Determining Cost-Effectiveness

Payers could simply not use QALYs when determining the cost-effectiveness of treatments or drugs at all. QALYs are only *one* possible outcome measure that researchers could use to determine the impact of a treatment on extension of life and quality of life.²²⁸ Cost-effectiveness studies could instead use other

measures that present fewer ethical problems, or simply are better at expressing the true benefit patients gain from treatment, than QALYs.

For example, the researcher could determine the number of individual cases of disease prevented, the number of deaths that were prevented, the number of years of life that were saved or would be saved, or any other possible benefit of the treatment. Payers could then evaluate whether this health outcome was worth the cost of the treatment.²²⁹ Ariel Beresniak provides an example where, for rheumatoid arthritis, if the benefit of the treatment is remission, the researcher could determine the “cost per clinical remission.”²³⁰ The use of cost-

[T]hey could use a cost-benefit analysis, which converts the health outcomes resulting from treatment into an amount of money and then subtracts that amount of money from the cost of the treatment.

effectiveness generally may still devalue clinically effective but high-cost treatments (such as, especially, cancer treatments),²³¹ which may harm individuals with disabilities and other chronic illnesses.

Instead of using a cost-effectiveness analysis, policymakers and researchers could also determine whether a treatment’s value outweighs its costs in some other way. For instance, they could use a cost-benefit analysis, which converts the health outcomes resulting from treatment into an amount of money and then subtracts that amount of money from the cost of the treatment.²³² For example, in a cost-benefit analysis, an insurer could determine how much money the insurer would save if a specific type of cancer were treated (as compared to the costs of hospitalization) and then subtract that amount of money from the cost of the cancer treatment.

There are still ethical concerns about the use of cost-benefit analysis in a healthcare context. One concern is that converting healthcare outcomes into money is a controversial idea that is often described as “putting a dollar value on life.” This is also similar to the idea of “cost per QALY,” which is also a way of putting a cost on a healthcare outcome and determining whether the cost is reasonable.²³³ Nonetheless, cost-benefit analysis is one of the more frequently used alternatives to cost-effectiveness analysis. Cost-benefit analysis is commonly used in non-healthcare sector contexts that still concern public health and wellness. For instance, the Environmental Protection Agency uses cost-benefit analyses when analyzing the impact of its environmental regulations. These regulations are analyzed primarily in terms of the degree to which they improve the health of the American public at large.²³⁴ The Environmental Protection Agency has experimented with the idea of using QALYs,²³⁵ but primarily uses cost-benefit analysis.²³⁶

Multi-Criteria Decision Analysis

Multi-criteria decision analysis (MCDA) is another alternative to QALYs that better acknowledges the complexity of healthcare decision-making. As explained by the Innovation and Value Initiative, MCDA allows decision-makers to simultaneously consider many different factors relevant to a healthcare decision (such as cost, clinical outcomes, and administrative burdens) and

determine how important each of these factors is to them.²³⁷

A payer using MCDA would first rank each factor that is relevant to the decision against one another.²³⁸ For instance, the decision-maker would determine whether clinical outcomes or cost matters more to them in a healthcare decision. Each of the criteria would then be given a weighted “score” representing that criteria’s importance to the decision-maker. Normally, when MCDA is used, there are a great many criteria that are being ranked in order of importance—sometimes as many as 15.²³⁹

Next, researchers would compare how each

MCDA allows decision-makers to simultaneously consider many different factors relevant to a healthcare decision (such as cost, clinical outcomes, and administrative burdens) and determine how important each of these factors is to them.

of the *treatments* being considered relate to one another. For example, Treatment A might have better clinical outcomes, but Treatment B costs less. Researchers would then create a score representing how each of the treatments fare with respect to each

of the criteria being considered. For example, Treatment A would receive a higher score for clinical outcomes than Treatment B, but a lower score for cost.

The next step is dependent on the decision that’s being made and the criteria that are being assessed, but when making a health care decision, it often involves generating a single average weighted score for each treatment that is the aggregate of both how the treatment scores on each of the criteria and how important those criteria are to the decision-maker, which then shows the relative value of the treatments to one another.²⁴⁰

MCDA has a variety of possible applications. For example, the Innovation and Value Initiative uses MCDA in its condition-specific model for rheumatoid arthritis. The model is intended to help a variety of different healthcare decision-makers determine the value of different anti-rheumatic (that is, anti-arthritis) drugs to them.²⁴¹ Importantly, the model can be altered to allow the decision-maker to consider how the drug will impact different subgroups of patients, such as subgroups of patients of a specific age, gender, severity of arthritis, etc.²⁴² As established in the section “QALYs Fail to Distinguish Between Subgroups of Patients with the Same Condition,” QALYs’ limited use for these purposes is a flaw of QALYs. Some stakeholders, such as some health economists, feel that use of MCDA is the most promising alternative to QALYs.²⁴³

MCDA does possess a number of flaws, the largest of these being ease of use. Researchers must accurately weigh what can be a large number of possible criteria accurately to make decisions. Additionally, according to Beresniak, many MCDA models are more complex than QALYs and may require a greater degree of expertise in order to be used.²⁴⁴ However, given that MCDA can be used to compare a wide variety of health-related criteria simultaneously—including both life extension, specific clinical benefits of treatment, and quality of life—a form of MCDA may represent the most likely effective alternative to the use of QALYs. NCD recommends that a condition-specific form of MCDA, with values based upon the perspectives of patients with the condition as seen in the Patient Perspective Value Framework, be utilized by payers to gauge the cost-effectiveness of treatments for the same condition.

Alternatives to QALYs That Use Primarily Patient Preferences

Patient rights groups believe that the best alternatives to QALYs allow patients with the condition being treated to define which treatments for the condition are of the highest “value,” and also what a “high-value” treatment is. While public and private insurers consider low-cost, clinically effective treatments to be of the highest value, patients may consider a wider variety of factors as important, such as the treatment’s impact on the ability to maintain relationships with one’s family and friends.²⁴⁵ Patient rights groups also argue that a good alternative to QALYs allows patients to evaluate the costs and benefits of a treatment across multiple areas of patients’ lives.

Patient Perspective Value Framework

FasterCures’ “Patient Perspective Value Framework” (PPVF) is a value framework that may satisfy PIPC’s criteria.²⁴⁶ While the PPVF has not yet been used extensively, FasterCures provides general examples of how the PPVF could be used in a number of situations, including by individuals as a decision-making aid and by public healthcare programs.²⁴⁷

The PPVF is divided into five broad “domains,” which are the five types of information patients usually consider when making healthcare decisions.²⁴⁸ These five domains are:

- **Domain 1: Patient Goals and Preferences,**
- **Domain 2: Patient-Centered Outcomes,**
- **Domain 3: Patient and Family Costs,**
- **Domain 4: Quality and Applicability of Evidence, and**
- **Domain 5: Usability and Transparency.**²⁴⁹

Information from Domains 1 through 4 is used by the decision maker to determine how valuable a drug or treatment is as compared to another drug or treatment, or multiple drugs or treatments, for the same condition. Researchers first attempt to determine what patients with the condition being treated value most in a healthcare treatment—that is, evidence for Domain 1. They then gather evidence related to: (1) Domain 2, which represents the health benefits and drawbacks of each intervention or drug for the patient; (2) Domain 3, the financial costs to the patient; and (3) Domain 4, how high-quality and comprehensive the evidence of a drug or treatment’s clinical effectiveness is. Domain 5 acts as a “foundation” for the other four Domains. A metric must be usable to be useful.

Researchers then weight the evidence from Domains 2, 3, and 4 based on the evidence they gathered for Domain 1, which is evidence of the goals and preferences of patients with the condition.²⁵⁰ PPVF then assigns a score to each treatment based on these calculations. PPVF’s assessment method appears similar to a form of multicriteria decision analysis, described further in the “Multicriteria Decision Analysis” section, which specifically considers matters of import to patients.

The PPVF uses “patient goals and preferences” to evaluate a far broader array of information about a treatment’s impact on patient quality of life than whether the treatment

extends life or has an impact on the specific, limited aspects of health-related quality of life typically measured by QALYs. For example, Domain 2, “Patient-Centered Outcomes,” uses patient preferences to evaluate the complexity of the treatment regimen and the treatment’s risks, side effects, and complications for patients.²⁵¹ This is a more realistic assessment of the myriad possible impacts a healthcare treatment can have on the lives of patients. The broader array of quality of life considerations would also prevent two treatments from receiving the exact same score, as no two treatments would have exactly the same impact on every single domain.

PPVF and similar methods can only be used to compare two different drugs or treatments for the same condition.²⁵² Payers could not use the PPVF to determine whether a drug for cystic fibrosis would be of higher value than a drug for hypertension. Some stakeholders feel that this

would not be a flaw at all, as it protects against many of the ethical issues that occur when QALYs are used to compare unlike treatments. The PPVF has never been used, however.²⁵³ It is therefore unclear how it would operate in practice.

The Efficiency Frontier

The German Institute for Quality and Efficiency in Health Care has adopted a method of assessing cost-effectiveness known as the efficiency frontier.²⁵⁴ Generally, an “efficiency frontier” in

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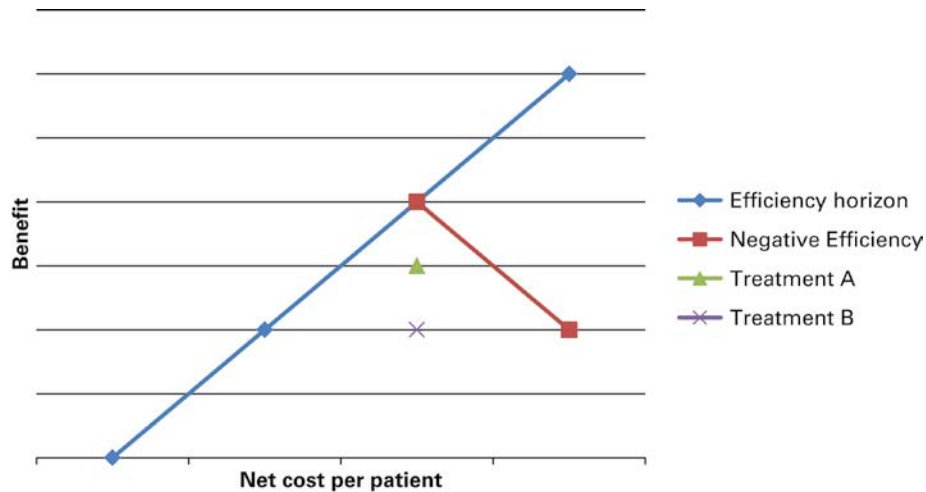


Figure 1. Example of an efficiency frontier.

Source: German Institute for Quality and Efficiency in Health Care.²⁵⁷

economics is the set of possible actions that offer either the greatest possible benefit for the cost involved or the lowest possible cost for the amount of benefit involved.²⁵⁵ A set of possible actions can be expressed as points on a scatter plot, and the “efficiency frontier” can be expressed as any of these points that line up with a line going through the center of the graph.²⁵⁶ Figure 1 is an example of an efficiency frontier.

The line going through the center of the graph is the efficiency frontier. The points on the graph represent, in the healthcare-specific example in Figure 1, treatments. The points along the line represent the most cost-effective options. While in an investment context, no points above the line could exist, in a healthcare context, they would represent healthcare treatments that are highly

cost-effective, or much more cost-effective than current approaches.²⁵⁸

The approach Germany proposed for evaluating healthcare treatments is to place the cost per patient on the x-axis (horizontal axis) of the graph, and the possible benefit on the y-axis (vertical axis) of the graph.²⁵⁹ The researcher

Generally, an “efficiency frontier” in economics is the set of possible actions that offer either the greatest possible benefit for the cost involved or the lowest possible cost for the amount of benefit involved.

would then add points to the graph representing different possible treatments for the same condition, and could use the resulting scatterplot to see which of these treatments is most cost-effective—such as

how cost-effective a new treatment would be as compared to current treatments.²⁶⁰

The graph format allows health economists to easily compare the costs and benefits of various interventions to one another. For example, in Figure 1, the “negative efficiency” line shows that

the hypothetical treatment represented by the red point closest to the blue line is clearly more cost-effective than the red point farther away. However, Treatment A provides slightly more benefits but costs more than the treatment on the blue line beneath it, though it is less cost-effective than the treatment on the line above it.

A researcher using an efficiency frontier could determine that the benefit of a lung cancer treatment was “restoring/maintaining lung function,” and determine a way to measure lung function in terms of percentages or numbers.²⁶¹ The researcher could also determine how much each lung cancer treatment would cost per patient per year. The researcher would then graph each lung cancer treatment along a scatter plot where “restoring/maintaining lung function” was the benefit on the y-axis, and cost per patient per year was along the x-axis. The researcher could then see visually which lung cancer treatments were the most efficient use of resources.

The main benefit of this approach is that it is clear, easy to use, and transparent. Additionally, it does not require the health economist to use QALYs as the measure of a treatment’s benefit.²⁶² The benefit on the graph could instead be the specific benefit that comes from the treatments, rather than an arbitrary number representing only some limited aspects of “quality of life” combined with the extent to which a treatment extends life. However, if QALYs are not used, it would only be possible to look at either one benefit of a healthcare treatment at a time, or different benefits that have been aggregated into a single number.²⁶³

Are There Alternatives to QALYs That Perform the Same Functions as QALYs?

QALYs continue to enjoy widespread use by health economists, researchers, and policymakers internationally and in the United States, despite the existence of alternatives. This is likely because, as multiple researchers have noted, QALYs are: (1) easy for policymakers to use (as they combine quality and quantity of life together and so payers would not need to determine how effective the drug is at improving quality and quantity of life separately); (2) well-established; and (3) allow policymakers to compare unrelated treatments to one another. As explained in the sections pertaining to each

alternative, no one alternative serves all of the functions of QALYs.

Many health economists have remarked that one of the reasons QALYs

persist despite their flaws is that there is no perfect replacement. These individuals have stated that while QALYs are imperfect at best, there are no sufficiently developed alternatives to QALYs and therefore QALYs remain “the best option available.”²⁶⁴ Other stakeholders disagree with this premise. Beresniak has argued that it is not sufficient, if QALYs lack scientific validity and do not measure what they claim to measure, to simply state that QALYs are the “best” option available, although he, too, says that no single alternative can act as a replacement.²⁶⁵

Some of the individuals NCD interviewed argued that *no* metric should serve all of the

Many health economists have remarked that one of the reasons QALYs persist despite their flaws is that there is no perfect replacement.

functions of QALYs, such as comparing unrelated treatments to one another.

Stramondo remarked,

I think it would be impossible to make judgments about how different technologies impact something as complex as quality of life. You could make a good judgment on Assistive Devices A and B assisting with the same function. Wheelchair A and B could be better or worse at assisting the same function. You could make comparisons among treatments with similar goals. The problem is when you want to compare an anti-nausea medication against a new stair-climbing wheelchair. How do you decide which one to fund based on which improves quality of life more? A concept like quality of life is so multidimensional, that's really tricky and probably impossible.²⁶⁶

Ne'eman stated something similar:

There's no reason why you must conflate life extension and disability mitigation into a single number. The only reason to do that is because they want a measure that can

be used across categories, [a measure] that can compare a cancer and a cystic fibrosis drug. If you don't require comparisons across categories, you can use diagnosis-specific measures. . . . I advocate saying, "Let's compare cancer drugs to other cancer drugs."²⁶⁷

Dr. Steve Pearson of ICER stated, "In my view, the current system is not working for patients, and [they're] being harmed every single day by the fact that the prices for drugs and treatments are so poorly aligned for their benefits." He believes it is "healthy for us to help force these questions into the forefront and have them in public as uncomfortable as they may be . . . [it is] important enough given the cost and the access problems . . . to try to do it in the open and [to] try to use evidence of cost-effectiveness as one important anchor [for] that discussion."²⁶⁸ Pearson's concerns are shared by many in the United States.

While these conversations are clearly necessary, it is not clear that QALYs are the best means of facilitating such conversations. There may be alternative means of incorporating "value" into healthcare coverage decisions.

Chapter 6: Recommendations

Congress

When enacting health reform bills, Congress should:

- Avoid creating provisions of any bill that would require the agency with management and oversight responsibilities (such as, for example, HHS) to cover only the most cost-effective drugs and treatments, or to require the agency to impose restrictions on less cost-effective treatments.

Congress should pass legislation:

- Prohibiting the use of QALYs by Medicaid and Medicare.
- Congress should provide funding to HHS for research on best practices on the use of cost-effectiveness to inform benefits and coverage decisions with respect to United States national health insurance programs, such as Medicare and Medicaid. “Best practices” in this case refers to a means of utilizing cost-effectiveness research that facilitates greater access to care and does not reduce access to care for people with chronic health conditions and disabilities.
- Congress should fund a report by the Government Accountability Office that examines how cost-effectiveness studies influence agency decision making, particularly cost-utility analysis (CUA) studies.

Department of Health and Human Services (HHS)

- HHS should consider including explicitly recruiting people with disabilities and chronic illnesses as members of committees and working groups formed to develop effective healthcare reform and strategies for lowering the cost of prescription drugs.
- HHS should support healthcare providers by issuing guidance on what steps to take if their patient's health insurance agency refuses to cover recommended treatment on the basis of that treatment's cost-effectiveness.

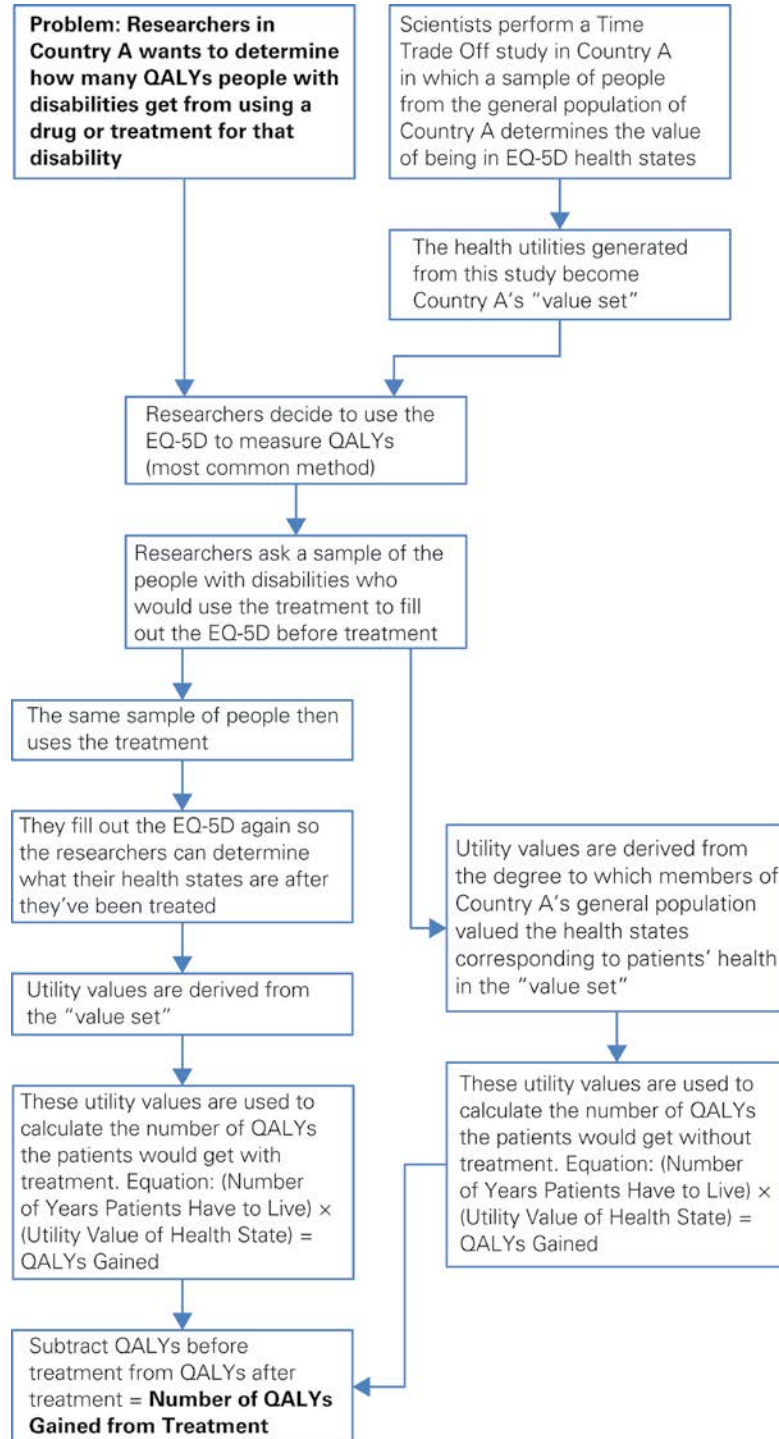
US Department of Health and Human Services (HHS) Office for Civil Rights (OCR); US Department of Justice (DOJ) Civil Rights Division

- DOJ and OCR should jointly issue guidance clarifying that the ADA applies to coverage programs that states operate, such as Medicaid.
- OCR, in consultation with DOJ as appropriate, should issue guidance to HHS sub-agencies, such as the Centers for Medicare & Medicaid Services as well as to State Medicaid Agencies, clarifying that:
 - Section 504 and Section 1557 also apply to Medicaid programs because they receive federal financial assistance. The guidance should specifically discuss how these authorities apply to benefits and reimbursement decisions, and that payment decisions should not rely on cost-effectiveness research or reports that are developed using QALYs; and
 - Section 504 and Section 1557 apply to health insurance programs operated by recipients of federal financial assistance from HHS. The guidance should discuss that covered health insurance programs should not rely on cost-effectiveness research or reports that gather input from the public on health preferences that do not include the input of people with disabilities and chronic illnesses.

HHS Centers for Medicare and Medicaid Services (CMS)

- CMS should utilize well-established alternatives to QALYs, such as Multicriteria Decision Analysis, which is a method that better acknowledges the complexity of healthcare coverage decisions, or cost-benefit analysis, when the exact benefits and costs of a drug or treatment are known. CMS could utilize these methods in combination, such as using cost-benefit analysis as one component of a Multicriteria Decision Analysis. If CMS does utilize cost-effectiveness analysis, it should consider utilizing it as one component of a condition-specific Multicriteria Decision Analysis.
- CMS should refrain from pursuing means of reducing Medicare and Medicaid prescription drug costs that attempt to model US pricing after the pricing in other countries, which may heavily rely on QALYs and often deny people with disabilities access to needed care.
- CMS should rescind the Advanced Notice of Proposed Rulemaking, which proposed an IPI for Medicare Part B.
- CMS should contribute to the development and use of value frameworks that utilize patient preferences to define which drugs and treatments are valuable, such as FasterCures' PPVF.

Appendix A: Calculation of QALYs Flowchart



Endnotes

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