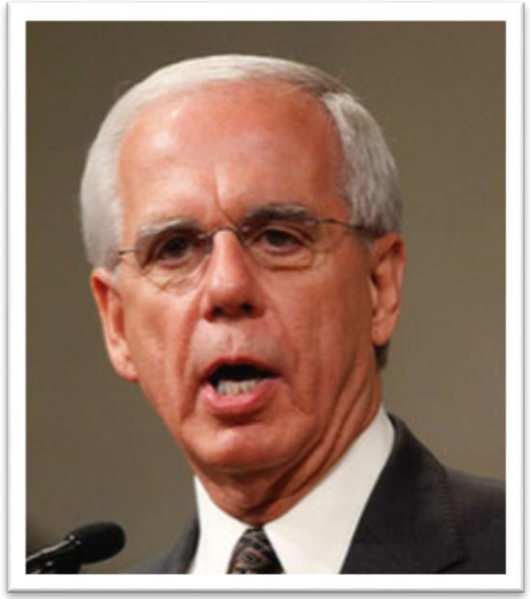
Value Assessments: The Perils of QALYs and Similar Metrics

October 25, 2019

PIPC

Honorable Tony Coelho, PIPC Chairman

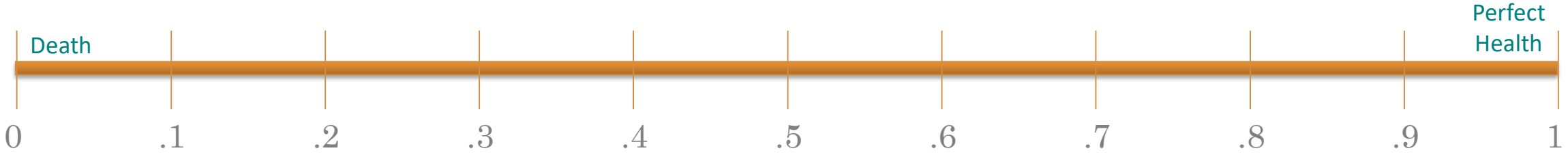
- Tony Coelho is a former United States Congressman from California and primary author and sponsor of the Americans with Disabilities Act
  - *"Nothing about us without us"*
- Tony is a person with epilepsy
- PIPC began as a coalition supporting PCORI's creation, with a voice and a vote for patients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



PIPC

QALYs discriminate against people with disabilities and serious chronic conditions by placing a lower value on their lives

What's the value of your life?



Person with Cancer

Person with Rheumatoid Arthritis

Person with Diabetes

QOL Improvement: QALY Challenges

- Under population survey models, the non-disabled population may systematically overestimate the burden of life with disability.
  - Research suggests a majority of American public says they would rather have HIV than be blind (Scott, 2016).
- Common QALY measure (EuroQol-5D) rates inflammatory arthritis as “worse than death” (Harrison, 2009).
  - Significant variation between TTO and VAS quality of life assessments reported under EuroQol-5D
- Under models where PWD self-report QoL, well supported people with disabilities who report relatively high levels of quality of life due to access to adequate support may find it very hard to demonstrate sufficient gains in QoL due to treatment efficacy.
- Exacerbate disparities by relying on RCTs that do not reflect subpopulations.

PIPC

The evLYG

ICER's future reports will incorporate more prominently a calculation of the Equal Value of Life Years Gained (evLYG), which evenly measures **any** gains in length of life, regardless of the treatment's ability to improve patients' quality of life.

In other words, if a treatment adds a year of life to a vulnerable patient population – whether treating individuals with cancer, multiple sclerosis, diabetes, epilepsy, or a severe lifelong disability – that treatment will receive the same evLYG as a different treatment that adds a year of life for healthier members of the community.

Supplementing the QALY, Not Replacing It

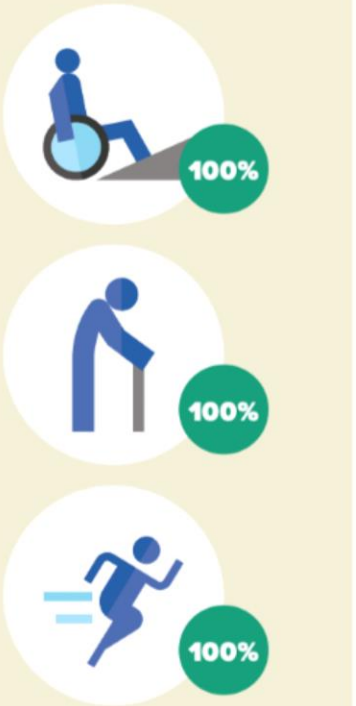
To maintain the ability of cost-effectiveness analyses to reflect the full benefits that treatments may have on quality of life, ICER will continue to calculate each treatment's QALY gained. The cost per QALY gained remains the best way for policymakers to understand how well the price of a treatment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

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

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

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

WITH evLYG, ONE ADDED YEAR = ONE ADDED YEAR



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

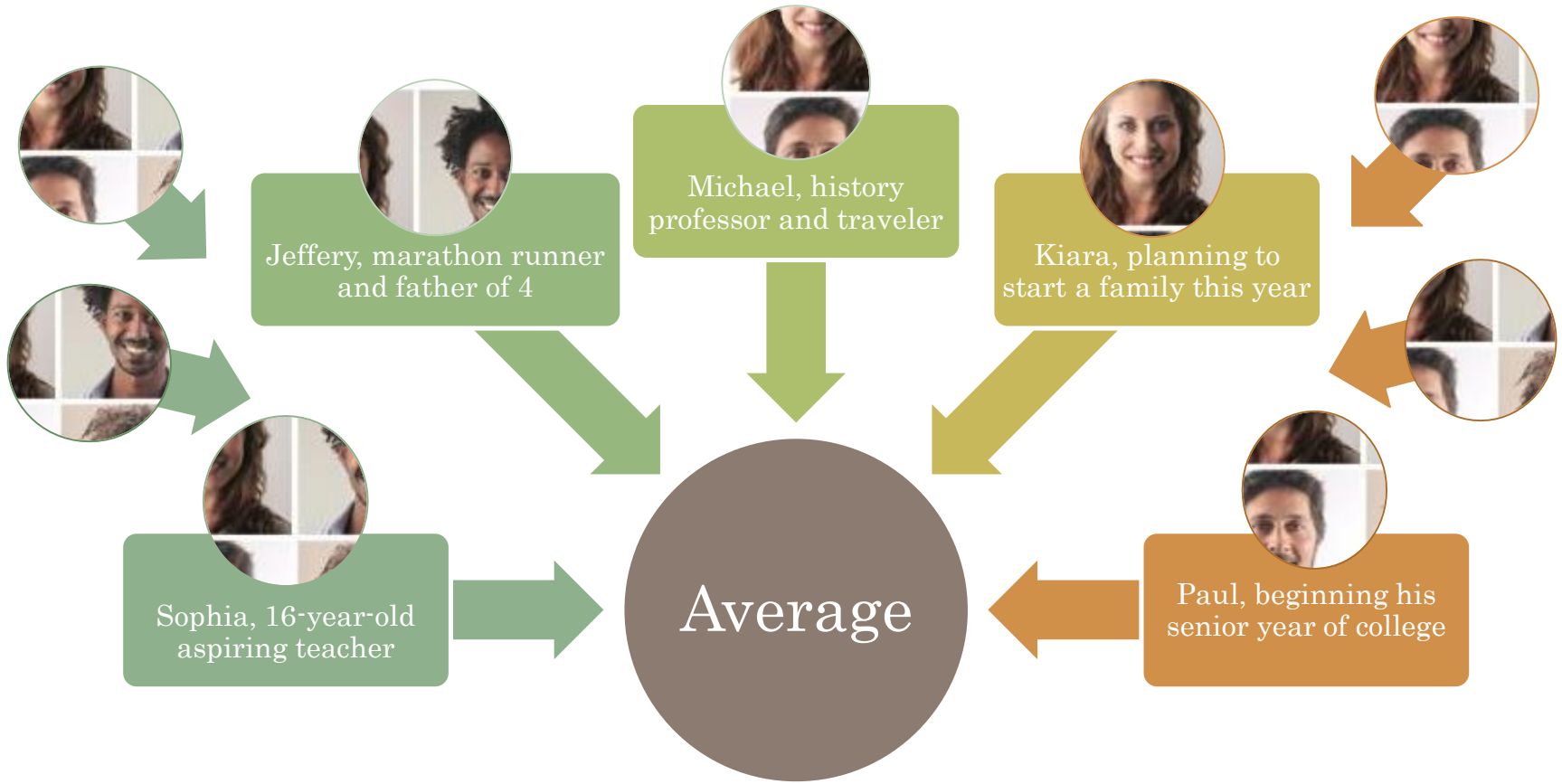
PIPC

Why the evLYG Doesn't Fix the Problem

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

PIPC

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



PIPC

Different People Respond Differently to the Same Drugs

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

Institute for Clinical Economic Review
ICER's Evolution

PIPC

VALUE OUR HEALTH

Flaws in ICER's Methods

- Reliance on Discriminatory Methods
  - Use QALYs and similar one-size-fits-all summary metrics.
  - Place a lower value on people with disabilities and serious chronic conditions
  - Sidesteps ethical problems related to using QALYs in health care decision-making.
- Failure to Meaningfully Engage Expert Stakeholders
  - Leaves patients, caregivers and clinicians who have firsthand experience with the condition under review out of the deliberation and voting process.
- Failure to Consider Outcomes that Matter to Patients and People with Disabilities
  - Values a treatment strictly from the health system and insurer perspectives. This can lead to situations where it is more "valuable" not to provide care for some patients because to do so would not be "cost-effective."
- Premature Assessments
  - Rush to deliver payers and policymakers value assessments immediately upon FDA approval has led to hasty reviews based on early assumptions, oversimplified models, and incomplete data.
- Lack of Transparency to Patients and People with Disabilities
  - Assessment process is a black box, leaving patients and people with disabilities in the dark on the assumptions used and important limitations that may have impacted the results.

PIPC

ICER Exacerbates Disparities

- Largely reliant on RCTs that do not reflect subpopulations
  - The risk profile of an average person is likely to be a proxy closely aligned to someone white, middle aged and male.
- Uses a population perspective (averages) for its cost-effectiveness modeling framework
  - No consideration of genetic background, demographics, risk and co-morbidities.
- Weights of health states translated into QALYs undertaken in predominantly white populations
- The selection and construction of the 'domains' that make up quality of life tools were constructed by a small group of elderly white men twenty years ago in Switzerland
- *Note:* ICER just started their review of Sickle Cell Disease treatments.
  - www.valueourhealth.org/sicklecell

PIPC

QALYs and Public Policy Implications

QALYs Have Historically Been Rejected by Policymakers

- The **ACA explicitly prohibits** PCORI from using the cost-per-QALY to determine effectiveness, and further restricts use in Medicare to determine coverage, reimbursement, or incentive programs.

- In 1992, **HHS rejected** Oregon's prioritized list of covered services for Medicaid citing the potential for violating the ADA due to use of QALYs and cost effectiveness.

PIPC

Oregon Health Plan

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

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

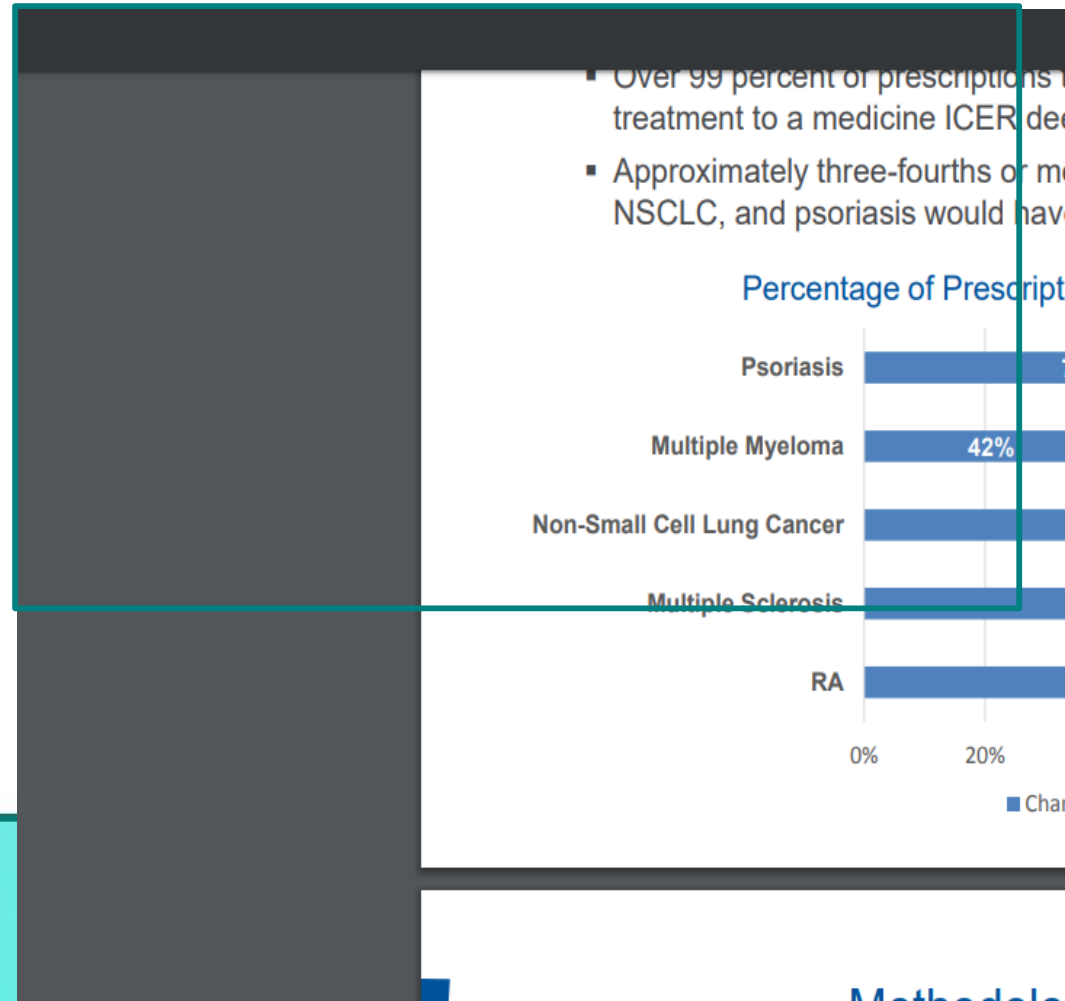
– Louis Sullivan, HHS Secretary, Letter in the New York Times, Aug 13, 1992

PIPC

Why do QALYs Matter?
Medicaid Access to Care!

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

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



IPI: Experience in Other Countries

Worse Outcomes

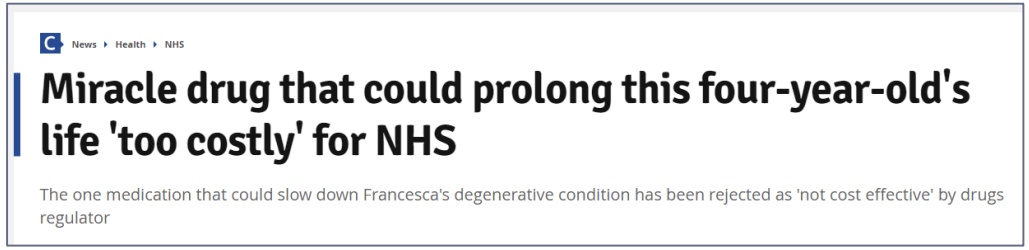
For breast, colon, lung and prostate cancers, 5-year survival rates are higher in U.S. than those in Canada, France, Germany, Italy, Japan and the U.K.

Fewer Options

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

Slower Access

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



News › Health › NHS

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

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

Cystic fibrosis sufferers denied life-prolonging drug by NHS

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

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

PIPC

New York

- New York: Passed legislation allowing for use of “value” assessments to determine supplemental rebates, also allowing for drugs with multiple in a class to be excluded from formularies.
- This year’s budget gave more explicit authority to use a third party like ICER to determine that “target” price.
- 40 advocacy groups sent a letter to the state legislature opposing the budget provision.

Developments in Other States

- The President's budget invites states to "make drug coverage decisions that meet state needs."
- CMS opened door to restricted coverage in their response to MA proposed waiver:
  - "Adopting a closed formulary with at least a single drug per therapeutic class would enable MassHealth to negotiate more favorable rebate agreements with manufacturers… the majority of commercial pharmacy benefit managers (PBMs) have adopted such closed formularies, which allow them to customize their drug offerings based on clinical efficacy and cost considerations."
- Massachusetts: Proposed policies modeled on New York reference "value"
  - Disability groups actively opposing and calling for a ban on use of QALYs
- California: The Legislative Analyst Office provided recommendations to consider the New York model.
- Oklahoma: ICER's QALY-based studies were used as part of deliberations to impose prior authorization requirements on Takhzyro, medication for Hereditary Angioedema, and Zolgensma, medication for spinal muscular atrophy.
- Tennessee: Wants flexibility to exclude breakthrough drugs from its formulary based on evidence of cost effectiveness.

Federal Activities

- H.R. 3 references international prices from 6 countries and authorizes use of studies from groups like ICER to determine comparative effectiveness of treatments.
- The Senate has discussed how to “pay for value.”
- The administration supports an international pricing index.

VALUE OUR HEALTH

Principles for Value Assessment

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

PIPC

www.valueourhealth.org

PIPC