Should Payers Increase Reimbursement for Treatments for Severe Diseases?

Empirical Approaches for Estimating Risk Preferences with Applications to Grace and Insurance Value

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Panelists

Jason Shafrin, PhD
Senior Managing Director
Center for Healthcare Economics and Policy,
FTI Consulting

Julia Thornton Snider, PhD
Senior Director, HEOR
Kite, A Gilead Company

Chris Skedgel, PhD
Director
Office of Health Economics

Disclosures

Jason Shafrin is an employee of FTI Consulting, a consulting firm that includes private sector, public sector and non-profit companies including numerous health care and life science entities

Julia Thornton Snider is an employee of Kite Pharma, A Gilead Company and holds Gilead equity.

Chris Skedgel is an employee of the Office of Health Economics, a registered charity and Independent Research Organisation, which receives funding from a variety of private and public sector sources.

Outline

Opening Remarks

Rationale for linking value to disease severity

- Julia Thornton Snider, PhD

Severity and risk aversion in HTA

- Chris Skedgel, PhD

Two alternative approaches for measuring value for severe diseases

- Jason Shafrin, PhD

Opening Remarks

Empirical evidence finds that willingness to pay for QALY gains is not constant across baseline disease severity levels

•**Scope insensitivity**. This assumes that individuals value QALY gains linearly. Thus, a QALY gain of 0.4 is valued twice as much as a QALY gain of 0.2 and four times as much as a QALY gain of 0.1

•**Severity independence**. This assumes that QALY gains of a given size are valued equally regardless of your initial health state. Thus if you have a QALY gain of 0.1, it assumes that gain is valued equally if you are paralyzed in a wheelchair or if you have some minor back pain. Numerically, it means that a QALY gain of 0.1 is the same if you have a baseline quality of life (QoL) of 0.25 as you would with a baseline QoL of 0.75 [where QoL is measured on a scale from 0 to 1]

- **Scope insensitivity often fails**:
  - *"WTP for a QALY (WTP-Q) gain of 0.1 was more than twice the WTP for the half-sized (0.05) QALY gain"*
- **Severity independence often fails**
  - *"Likewise, the 'severity' coefficient was found to be positive and significant implying that for two equally sized health gains, a QALY gain is valued higher in the more inferior health state (22222) than in the less severe health state (21121)."*

TABLE 4. Regression results (using a log-linear specification with clustered standard errors at the individual level)

	Model 1 (Baseline) Coef.(std. error)	Model 2 (R1) Coef.(std. error)	Model 3 (R2) Coef.(std. error).	Model 4 (R3) Coef.(std. error).	Model 5 (R1-R3) Coef.(std. error).
Scope	0.14 (0.06)***	−0.36 (0.02)***	1.07 (0.1)***	0.32 (0.08)***	0.15 (0.04)***
Severity	0.28 (0.08)***	0.15 (0.03)***	0.3 (0.13)**	0.24 (0.12)*	0.12 (0.07)*
Constant	7.21 (0.12)***	9.31 (0.06)***	5.36 (0.2)***	7.37 (0.16)***	8.92 (0.11)***
Respondents	1092	765	507	530	182
Observations	4368	3060	2028	2120	728

*,**,***Significant at 0.1, 0.05 and 0.01 levels, respectively.

Nielsen JS. Health Economics. 2021 May;30(5):923-31.

…leading some HTAs to adjust the value of QALYs and the development of other methods such as generalized risk-adjusted cost effectiveness (GRACE)

Health Technology Assessment With Diminishing Returns to Health: The Generalized Risk-Adjusted Cost-Effectiveness (GRACE) Approach

Darius N. Lakdawalla, PhD, Charles E. Phelps, PhD

Relative Health Loss (ℓ^*)	Relative Risk Aversion in Health (r_H^*)					
	0	0.25	0.5	0.75	1	1.25
0	1	1	1	1	1	1
0.1	1	1.03	1.05	1.08	1.11	1.13
0.3	1	1.09	1.2	1.31	1.43	1.56
0.5	1	1.19	1.41	1.62	2	2.38
0.7	1	1.35	1.83	2.47	3.33	4.5
0.9	1	1.78	3.15	5.61	10	17.7

NICE National Institute for Health and Care Excellence

QALY weight	Proportional QALY shortfall	Absolute QALY shortfall
1	Less than 0.85	Less than 12
x1.2	0.85 to 0.95	12 to 18
x1.7	At least 0.95	At least 18

Incremental QALYs gained (per patient using lifetime horizon)	Weight
Less than or equal to 10	1
11 to 29	Between 1 and 3 (using equal increments)
Greater than or equal to 30	3

Lakdawalla DN, Phelps CE. Value in Health. 2021 Feb 1;24(2):244-9.
NICE health technology evaluations: the manual. 31 Jan 2022: https://www.nice.org.uk/process/pmg36/resources/nice-health-technology-evaluations-the-manual-pdf-72286779244741

Rationale for linking value to disease severity

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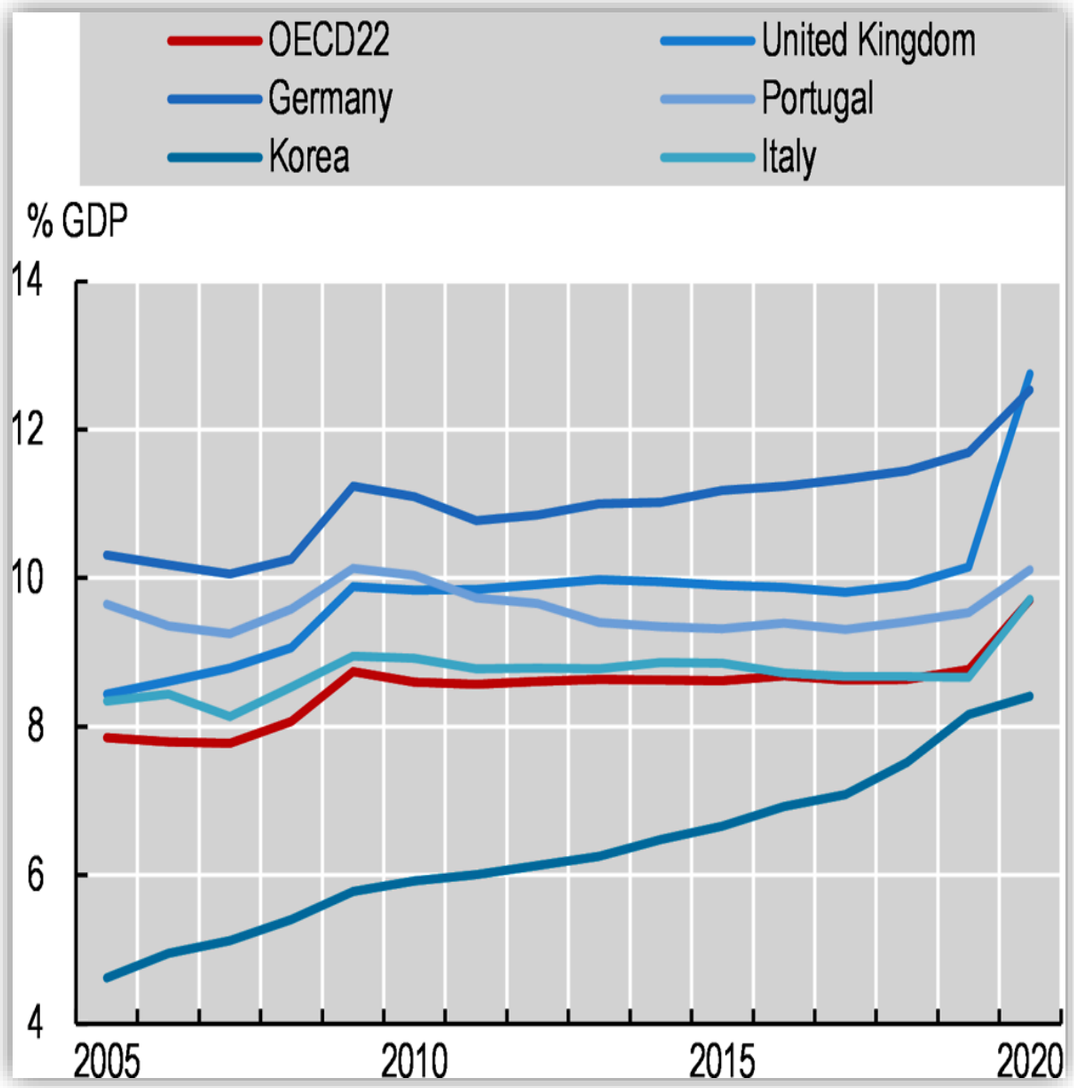
Rising healthcare costs can lead to tradeoffs in coverage decisions

- **Healthcare spending rising relative to GDP around the world**
  - 2020 is an outlier due to COVID, but still there is a broader trend for an increased share of the economy going to health plans.
  - Approving new therapies can increase budget impact
- **Health technology assessment (HTA) has arisen as tool for aligning healthcare spending with value and controlling budgets**
  - Tradeoffs are inevitably involved
  - For instance, cover more therapies or focus on covering therapies more generously for the most severe disease
- **Even in countries without formal HTA processes, coverage decisions must still be made**
  - Policies and costs to patients will influence access and uptake



OECD: Figure 7.3. https://www.oecd-ilibrary.org/sites/e26f669c-en/index.html

HTA reimbursement decisions typically hinge on value measured in quality-adjusted life years

HTA links reimbursement to value

- Health Technology Assessment (HTA) traditionally determines reimburse based on the incremental cost and health gains
- Health gains measured relative to using incremental quality-adjusted life-year (QALY) vs. standard of care

Most commonly used notion of value is ICER

- $ICER = \frac{Cost_{new} - Cost_{old}}{QALY_{new} - QALY_{old}} = \frac{\Delta Cost}{\Delta QALY}$

Standard approach assumes QALY gains valued the same across disease

- In this formulation, gaining a QALY from improving acne is as worthwhile to society as gaining a QALY from extending survival for patients with terminal illness

Do people view gains in QALYs the same regardless of the population or disease considered?

Coverage decisions and denials can generate controversy

Former UK Prime Minister David Cameron

"When I became Prime Minister three years ago, many patients with rare cancers were being denied lifesaving treatments. That is why we created the Cancer Drugs Fund"[1]

— "The funding of drugs rejected by NICE raises a fundamental ethical dilemma: should certain NHS patients' lives be valued more highly than others? In effect, the CDF undermines the underlying NICE/NHS principle that all lives are of equal value regardless of disease or any other patient characteristic."[2]

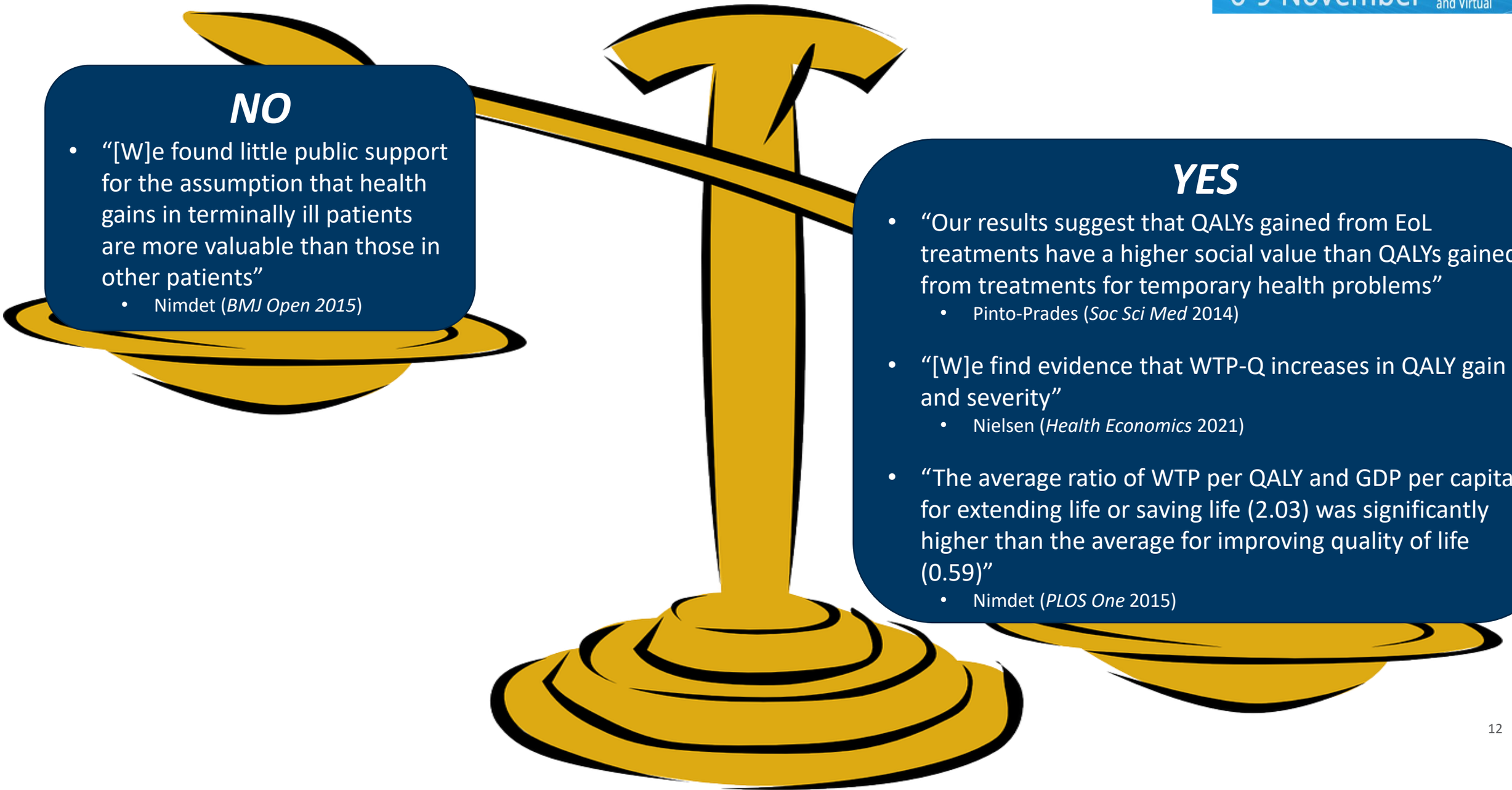
— "In many respects, the extensive coverage of the Cancer Drugs Fund closely mirrors a major media preoccupation with cancer. Although cancer is responsible for only 21% of disability-adjusted life years in the UK, there are many more newspaper stories about research on cancer than about the other main causes of the UK disease burden, cardiovascular disease (including stroke) and mental disorders.[3]

- **Woman Was Denied a Mammogram at Age 30 — but Ended Up with Stage 4 Breast Cancer: 'I Was Failed by the System'[4]**

- **"High fives and sobs greet UnitedHealthcare's reversal of denials for gene therapy"[5]**

1. https://www.gov.uk/government/news/thousands-of-patients-to-benefit-from-400-million-cancer-package
2. https://www.kingsfund.org.uk/blog/2014/09/cancer-drugs-fund-inequitable-and-inefficient
3. https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6194957/
4. https://people.com/health/woman-denied-mammogram-age-30-ended-up-stage-4-breast-cancer-philecia-labounty/
5. https://www.washingtonpost.com/business/economy/high-fives-and-sobs-greet-unitedhealthcares-reversal-of-denials-for-child-gene-therapy/2019/07/18/8ddeb3ae-a974-11e9-9214-246e594de5d5_story.html

Do people put greater value on QALYs gained treating severe disease?



NO

- "[W]e found little public support for the assumption that health gains in terminally ill patients are more valuable than those in other patients"
  - Nimdet (*BMJ Open 2015*)

YES

- "Our results suggest that QALYs gained from EoL treatments have a higher social value than QALYs gained from treatments for temporary health problems"
  - Pinto-Prades (*Soc Sci Med* 2014)
- "[W]e find evidence that WTP-Q increases in QALY gain and severity"
  - Nielsen (*Health Economics* 2021)
- "The average ratio of WTP per QALY and GDP per capita for extending life or saving life (2.03) was significantly higher than the average for improving quality of life (0.59)"
  - Nimdet (*PLOS One* 2015)

This controversy has led to calls for including additional aspects of value in HTA and broadening the approach to cost-effectiveness in particular

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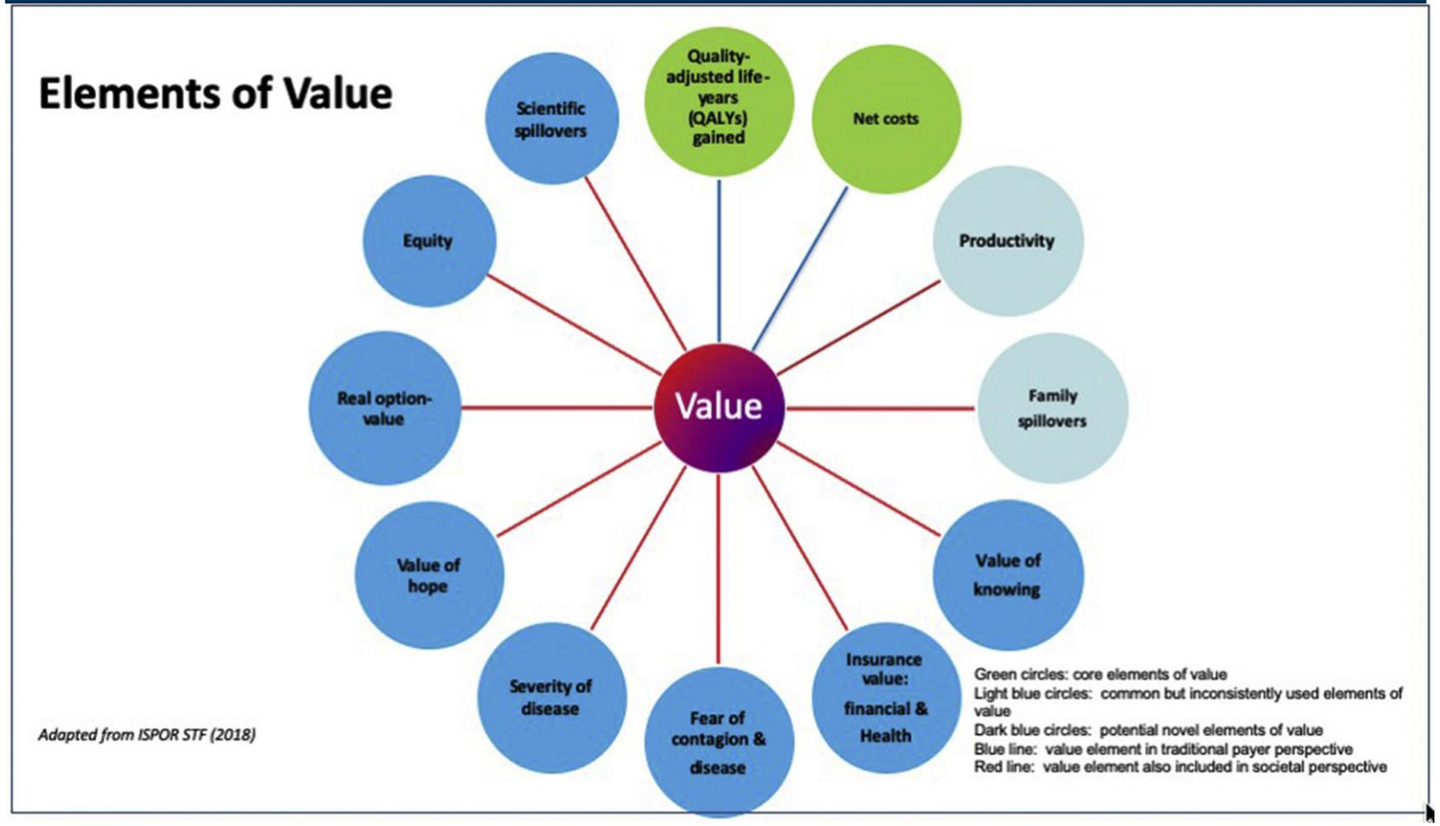
ISPOR Value Flower[1]



Therapies could have additional value if they

- Extend life
- Improve quality of life
- Reduce healthcare costs
- Increase productivity for the patient or caregivers
- Increase hope of long-term survival
- Allow time to survive until the next breakthrough
- Reduce inequities
- Treat severe diseases

GRACE:

- Some of these considerations can be taken into account in the generalized risk adjusted cost effectiveness framework[2]

1. Neumann. PJ. Value Health, 21 (2) (2018), pp. 119-123
2. Lakdawalla, Darius N., and Charles E. Phelps. *Value in Health* 24.2 (2021): 244-249.

Severity and risk aversion in HTA

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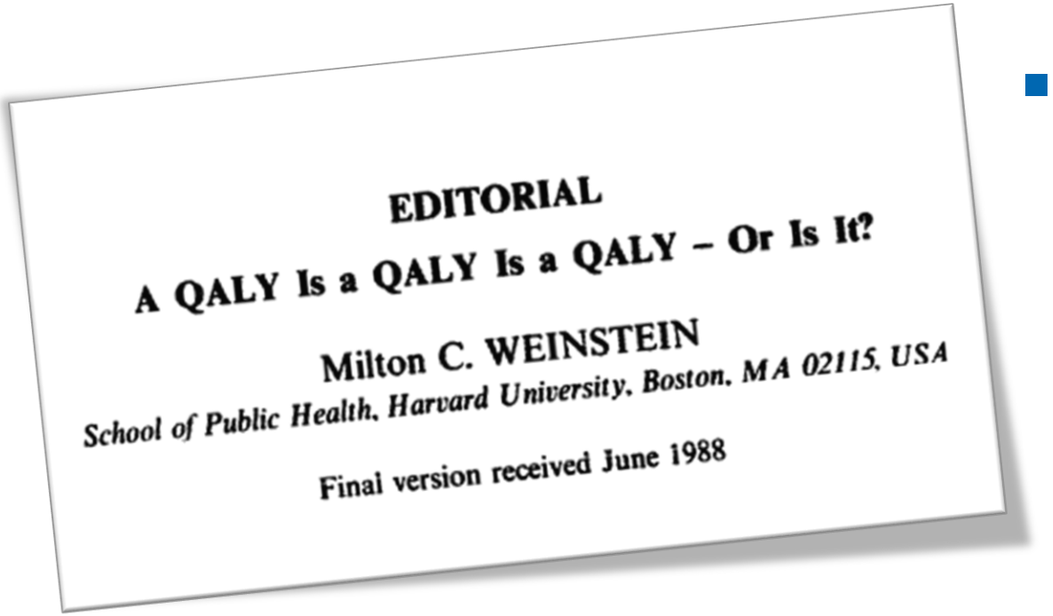
Equity and efficiency in HTA



EDITORIAL

A QALY Is a QALY Is a QALY – Or Is It?

Milton C. WEINSTEIN
School of Public Health, Harvard University, Boston, MA 02115, USA

Final version received June 1988

- Conventionally, health economics and HTA have treated health gains to different patients as interchangeable in terms of value, regardless of patient characteristics.

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Policy Perspective

Considering Severity in Health Technology Assessment: Can We Do Better?

Chris Skedgel, PhD, Nadine Henderson, MSc, Adrian Towse, MA, MPhil, David Mott, PhD, Colin Green, PhD

ABSTRACT

There is strong evidence that individuals and the public assign relatively greater value to health gains from relatively more severe health states. This preference is increasingly reflected in health technology assessment, with some consideration of severity incorporated by health technology assessment bodies in, among others, The Netherlands, England and Wales, Norway, Sweden, and the United States. If a societal "severity premium" is to be considered fairly and consistently, we argue that a more explicit and quantitative approach is needed. We highlight drawbacks of categorical approaches, especially discontinuities between severity categories that arguably violate concepts of vertical equity, and argue that a more continuous approach to understanding severity is needed. We also note challenges to more explicit approaches, including implications of a lower threshold for less severe conditions and the relative complexity of calculating a continuous severity adjustment.

Keywords: health technology assessment, priority setting, public preferences, severity.

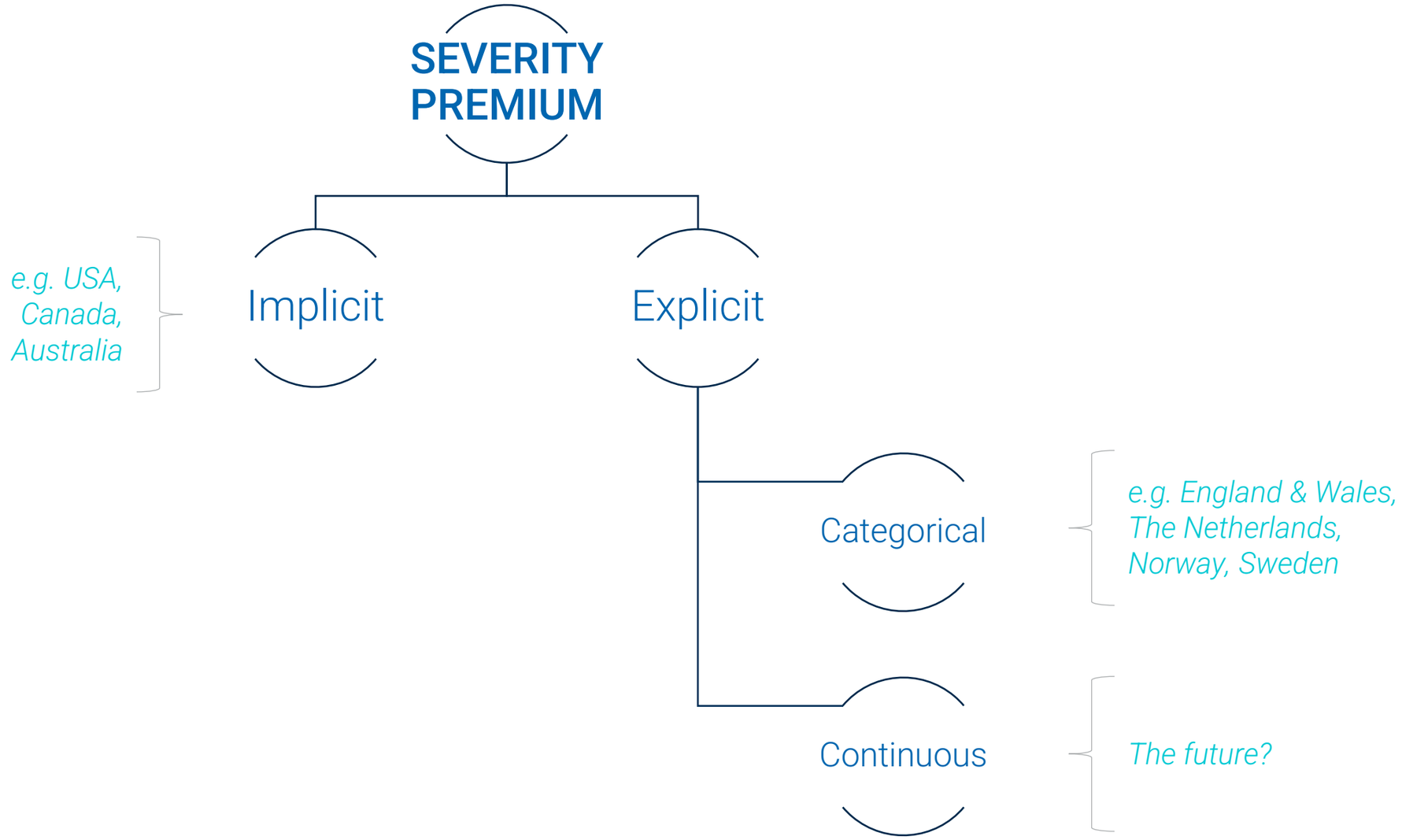
VALUE HEALTH. 2022; 25(8):1399–1403

- There is, however, a growing body of evidence that shows society is willing to sacrifice some efficiency to achieve a fairer or **more equitable** distribution of healthcare resources, particularly over **disease severity**.

“Underlying all [NICE] decisions is one fundamental social value judgement: *that advice from NICE to the NHS should embody values that are generally held by the population of the NHS*.

RAWLINS AND CULYER (2004)

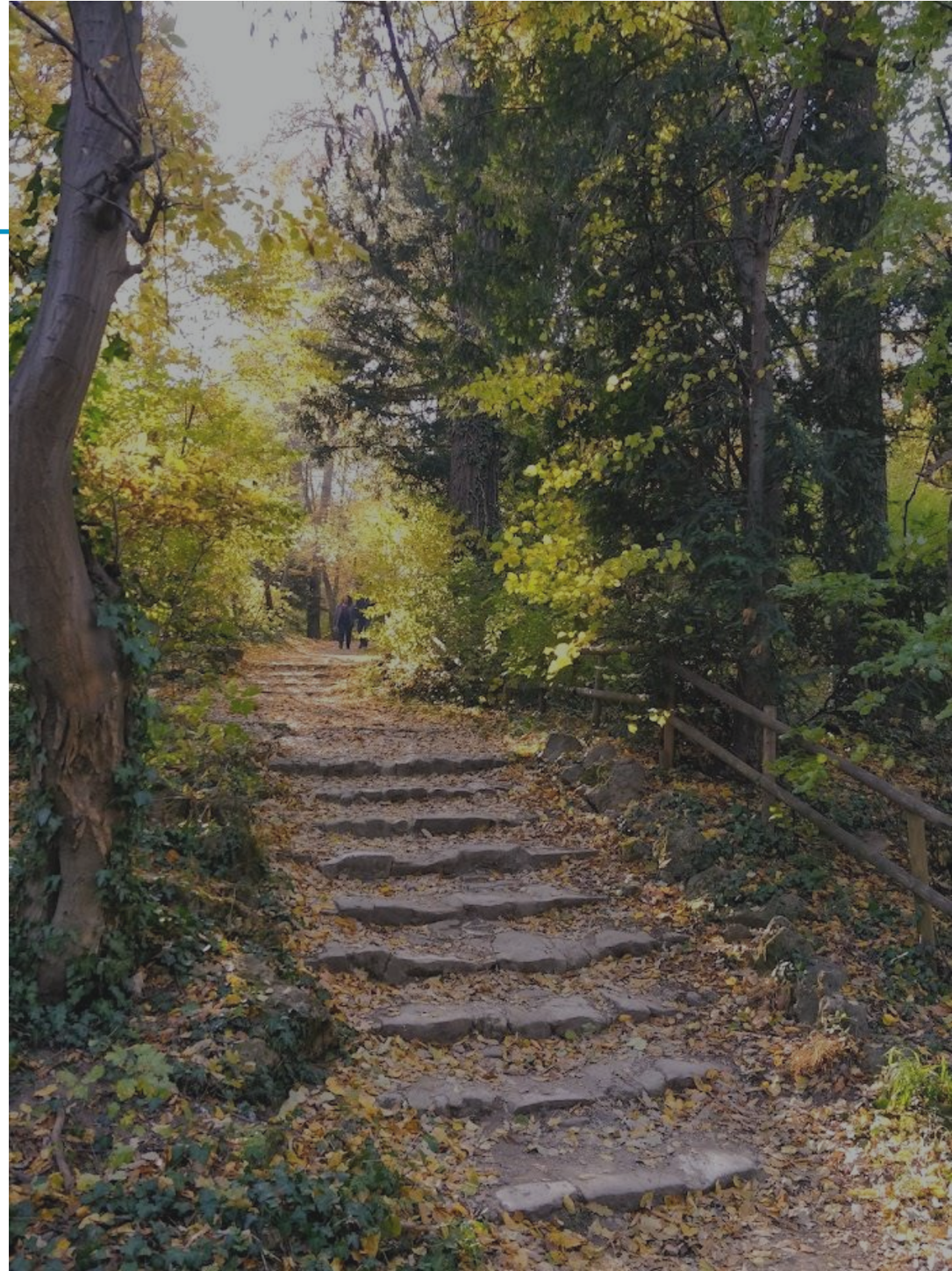
“Severity premiums” in national practice



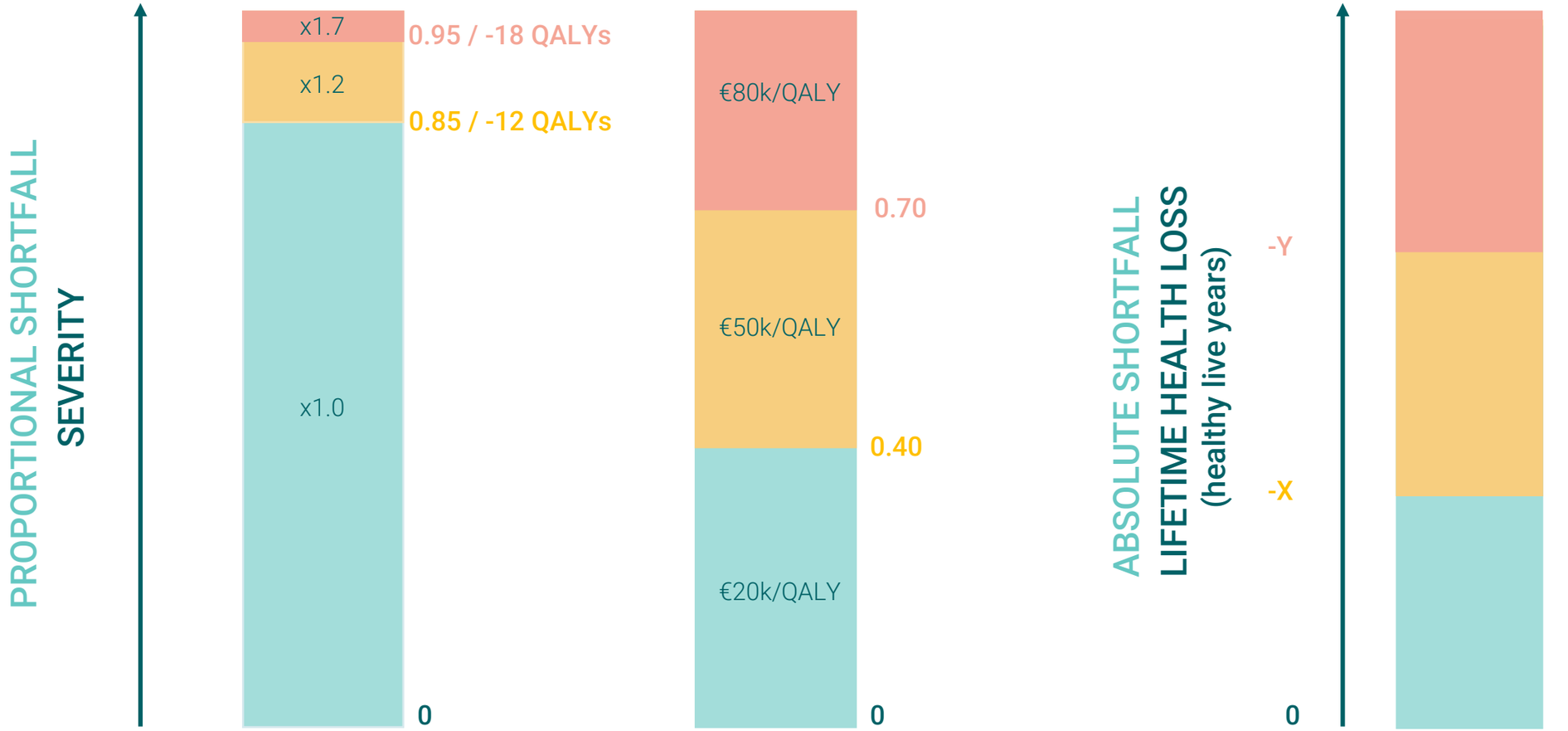
Categorical approaches to severity value adjustment

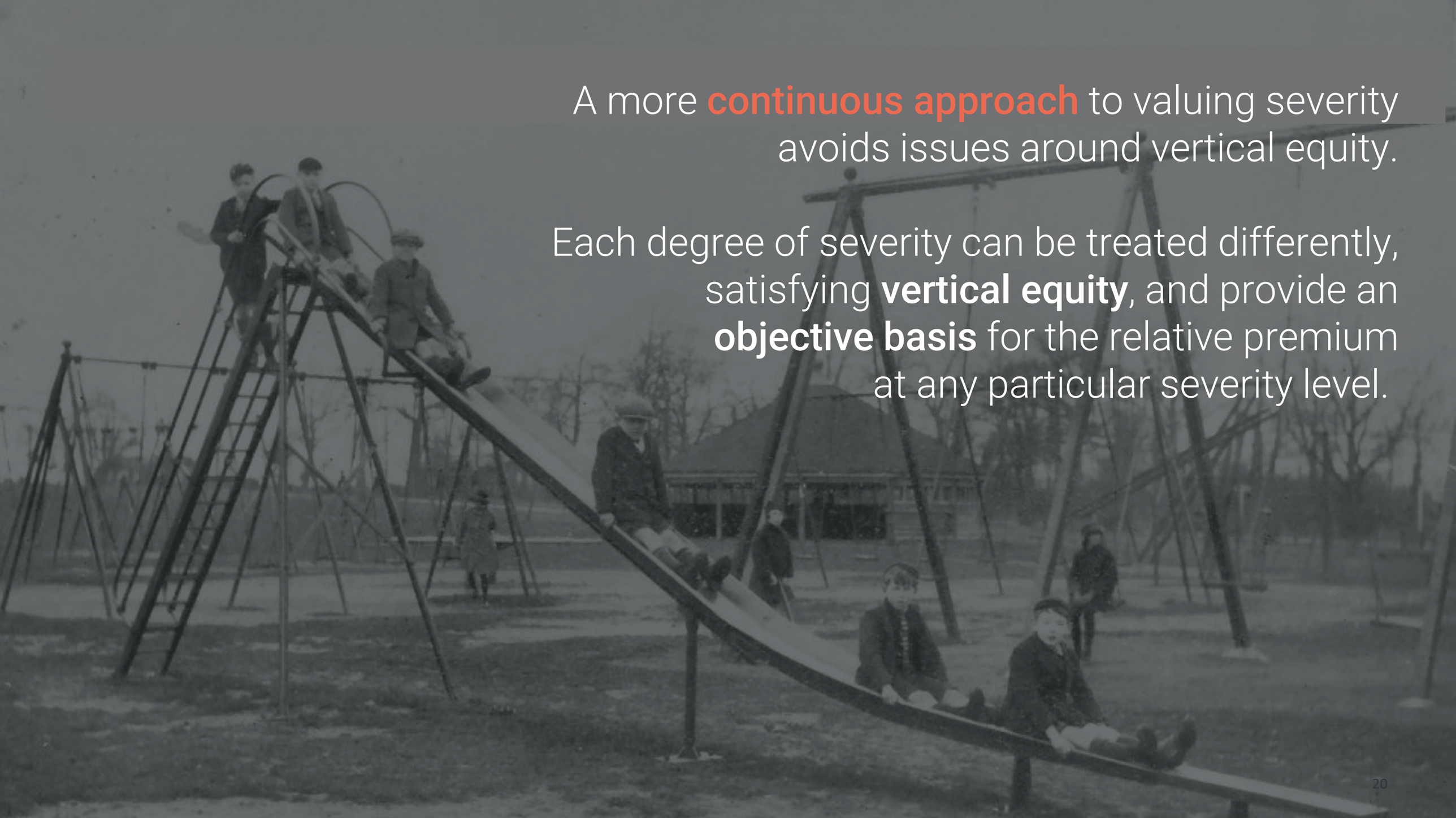
Under a **categorical approach**, different severity intervals are associated with a specific severity adjustment or premium.

This ‘step-wise’ approach is relatively simple to implement, but has potential drawbacks in terms of **vertical equity**.



Categorical approaches to severity value adjustment





A more **continuous approach** to valuing severity avoids issues around vertical equity.

Each degree of severity can be treated differently, satisfying **vertical equity**, and provide an **objective basis** for the relative premium at any particular severity level.

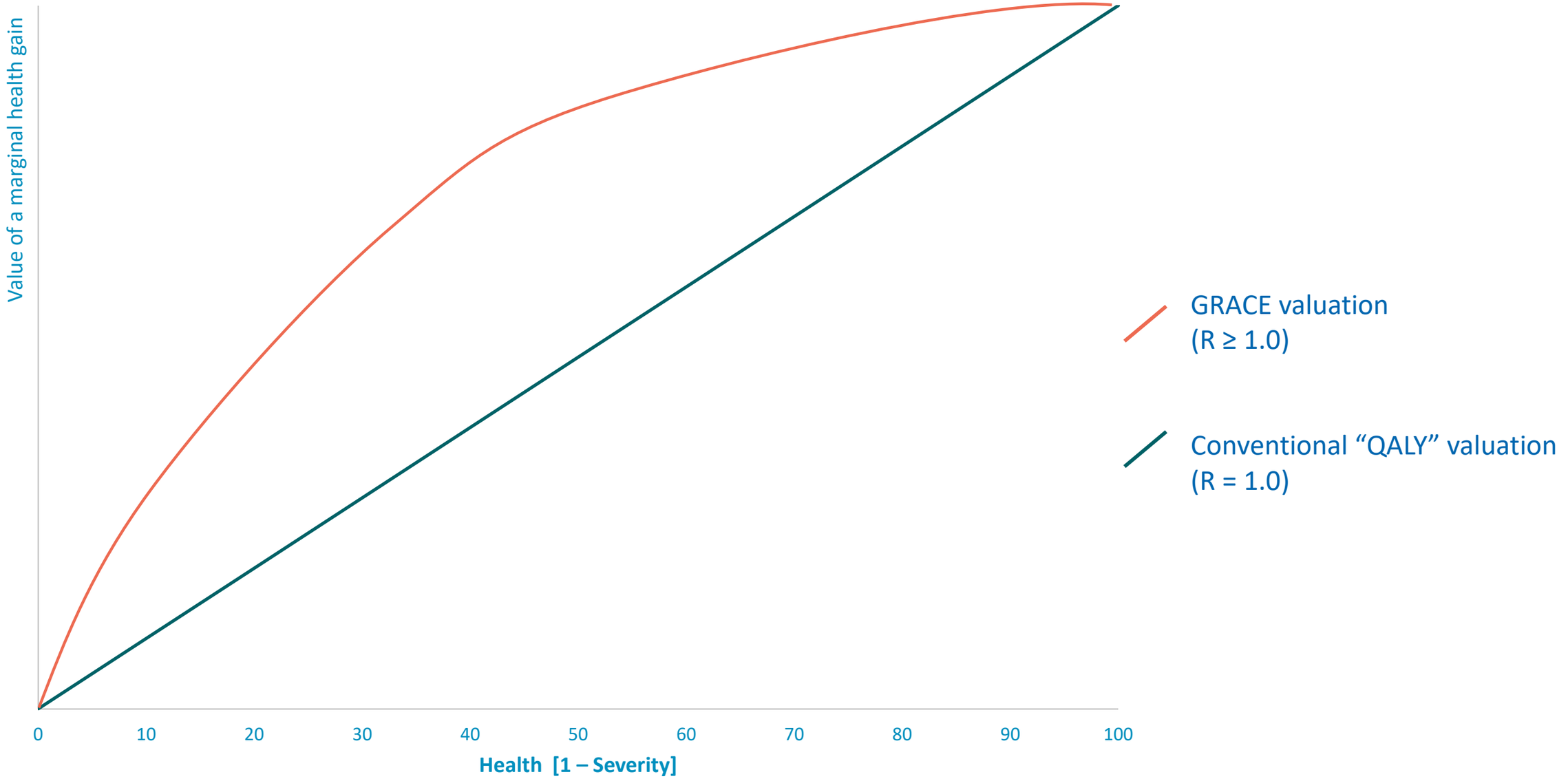
"Diminishing returns to health" in the GRACE model

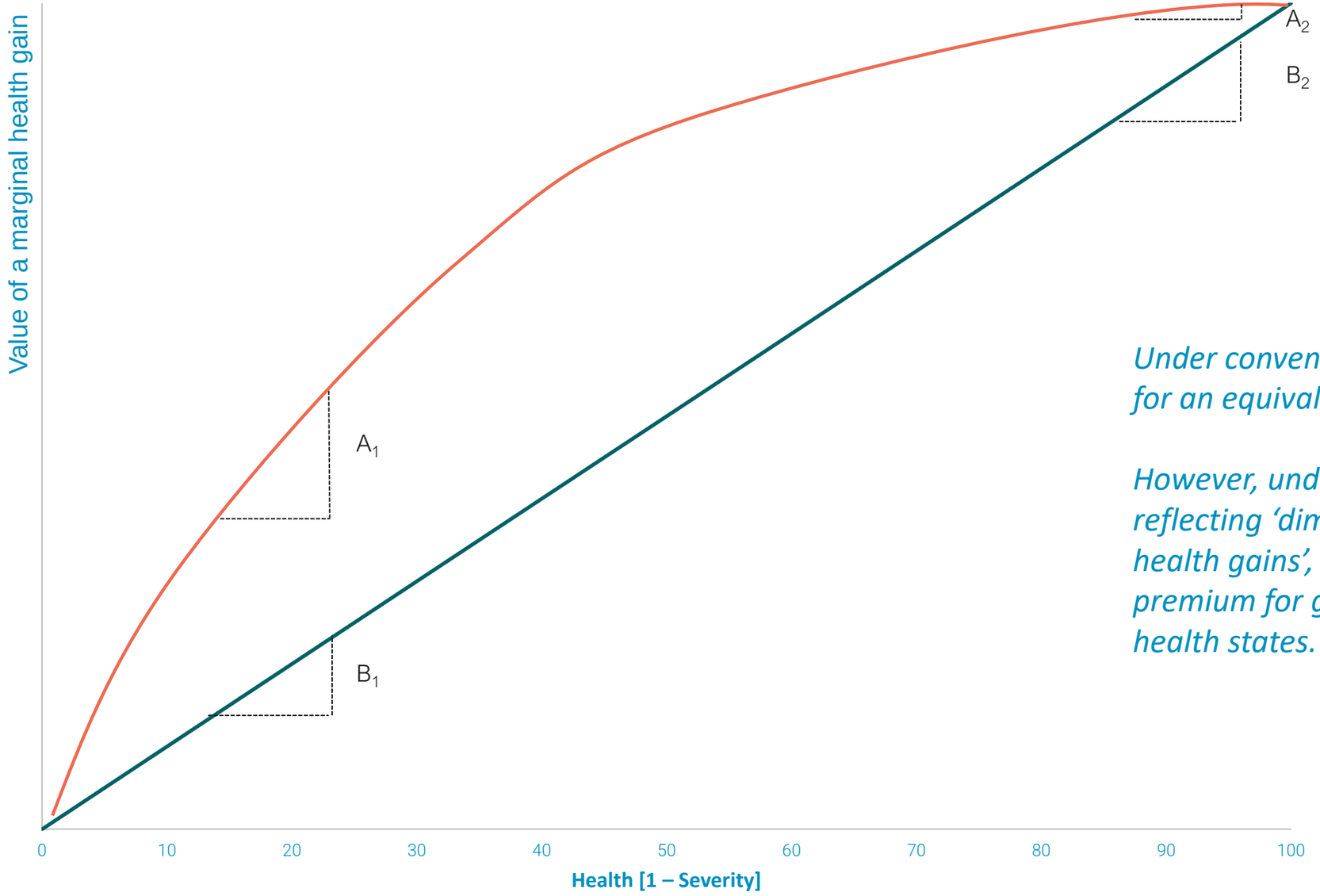
- Starting from the conventional cost per QALY model, Lakdawalla & Phelps (2022) propose a multiplier, ***R***, that represents the ratio of the marginal value of a health gain in a "severe" health state and in (almost) full health:

$$R = \frac{\partial U(\text{Severe})}{\partial U(\text{Healthy})}$$

- Under the conventional QALY model, R=1.0 ("A QALY is a QALY is a QALY")
- Under GRACE, R~1.0 for minor illness, but may be substantial (R>>1.0) for very severe conditions. This is applied to the acceptable cost-effectiveness threshold (CET) so that the decision rule becomes:

$$\frac{\Delta C}{\Delta E} \leq (CET \cdot R)$$


Value of a marginal health gain
0
10
20
30
40
50
60
70
80
90
100
Health [1 – Severity]
GRACE valuation
(R ≥ 1.0)
Conventional "QALY" valuation
(R = 1.0)



Under conventional valuation, $B_1 = B_2$ for an equivalent change in health.

However, under GRACE, $A_1 >> A_2$, reflecting 'diminishing returns to health gains', or effectively, a severity premium for gains from more severe health states.

Two alternative approaches for measuring value for severe diseases

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Discussion will focus on 2 topics

Measuring ***disease-specific risk aversion*** to implement GRACE

Measuring **insurance value** directly using stated preference surveys

Which treatment option do you prefer?

A risk averse person would generally prefer **Treatment A**

Health State		Treatment A	Treatment B
Healthy		0%	**25%**
Walk with a cane		**100%**	**50%**
Wheelchair		0%	**25%**

Measuring disease specific risk aversion over health states requires measuring utility by individual over health states

While **Person 1** has a relatively high utility of being in a wheelchair…

Health State	Person 1 Utility	Treatment A	Treatment B
Healthy 	1.00	0%	**25%**
Walk with a cane 	0.80	**100%**	**50%**
Wheelchair 	**0.68**	0%	**25%**
Expected utility		0.80	**0.82**

Measuring disease specific risk aversion over health states requires measuring utility by individual over health states

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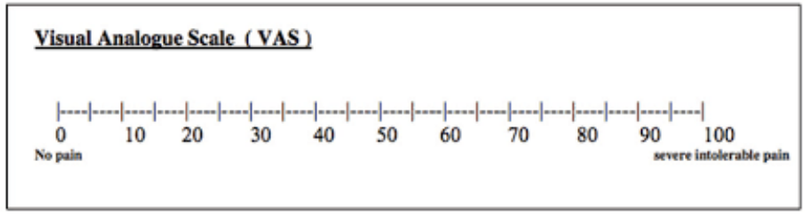
Person 2 has much lower utility level of being in in a wheelchair…

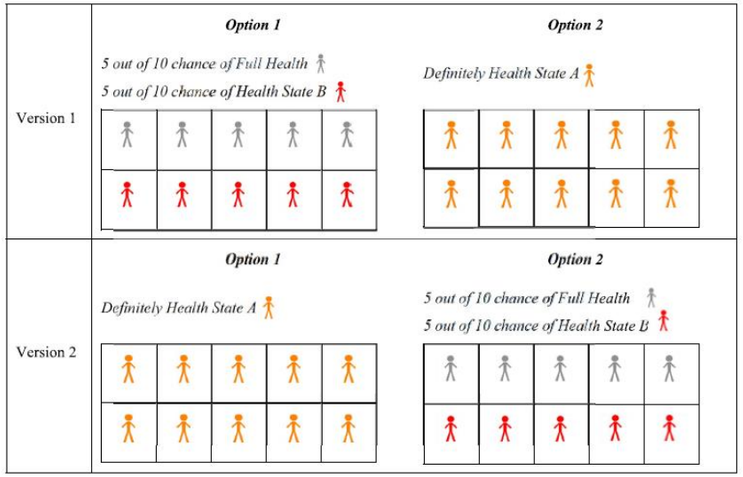
Health State	Person 2 Utility	Treatment A	Treatment B
Healthy 	1.00	0%	**25%**
Walk with a cane 	0.80	**100%**	**50%**
Wheelchair 	**0.20**	0%	**25%**
Expected utility		**0.80**	**0.70**

4-step approach to calculate disease-specific risk aversion for implementing GRACE

- Solicit health state utilities over health states by individual
- Measure risk preferences over different health states
- Calculate relative risk aversion based on (i) individual and (ii) aggregate utilities
- Implement GRACE methodology



ID	Treatment A				Treatment B				Your Choice	
	P	Days in full health	P	Days in full health	P	Days in full health	P	Days in full health	A	B
1	10%	**200**	90%	**160**	10%	**385**	90%	**10**	A	B
2	20%	**200**	80%	**160**	20%	**385**	80%	**10**	A	B
3	30%	**200**	70%	**160**	30%	**385**	70%	**10**	A	B
4	40%	**200**	60%	**160**	40%	**385**	60%	**10**	A	B
5	50%	**200**	50%	**160**	50%	**385**	50%	**10**	A	B
6	60%	**200**	40%	**160**	60%	**385**	40%	**10**	A	B
7	70%	**200**	30%	**160**	70%	**385**	30%	**10**	A	B
8	80%	**200**	20%	**160**	80%	**385**	20%	**10**	A	B
9	90%	**200**	10%	**160**	90%	**385**	10%	**10**	A	B



Arrieta A. *Health Economics*. 2017 Dec;26:97-113.
Yang M. *Health economics*. 2022 May;31(5):836-58.

Discussion will focus on 2 topics

Measuring ***disease-specific risk aversion*** to implement GRACE

Measuring **insurance value** directly using stated preference surveys

State preference methods can also be used to estimate the additional value from severe disease

Table 1. Approach to value decomposition.

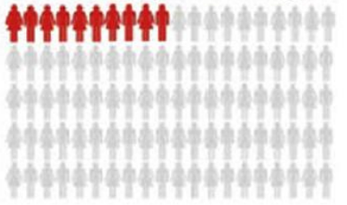
Value	Formula
Ex post conventional value of health gains for patients with lung cancer (ex post)	$[WTP_{cancer} - C]$
Ex ante conventional value of health gains for healthy people (ex ante)	$[p \times WTP_{cancer} - p \times C]$
Total value for healthy people	$[WTP_{atrisk} - p \times C]$
Incremental value of generous insurance as measured by the incremental value of risk reduction for healthy people	$[WTP_{atrisk} - p \times WTP_{cancer}]$

WTP indicates willingness to pay.

Shafrin J. *Value in Health*. 2021 Jun 1;24(6):855-61.

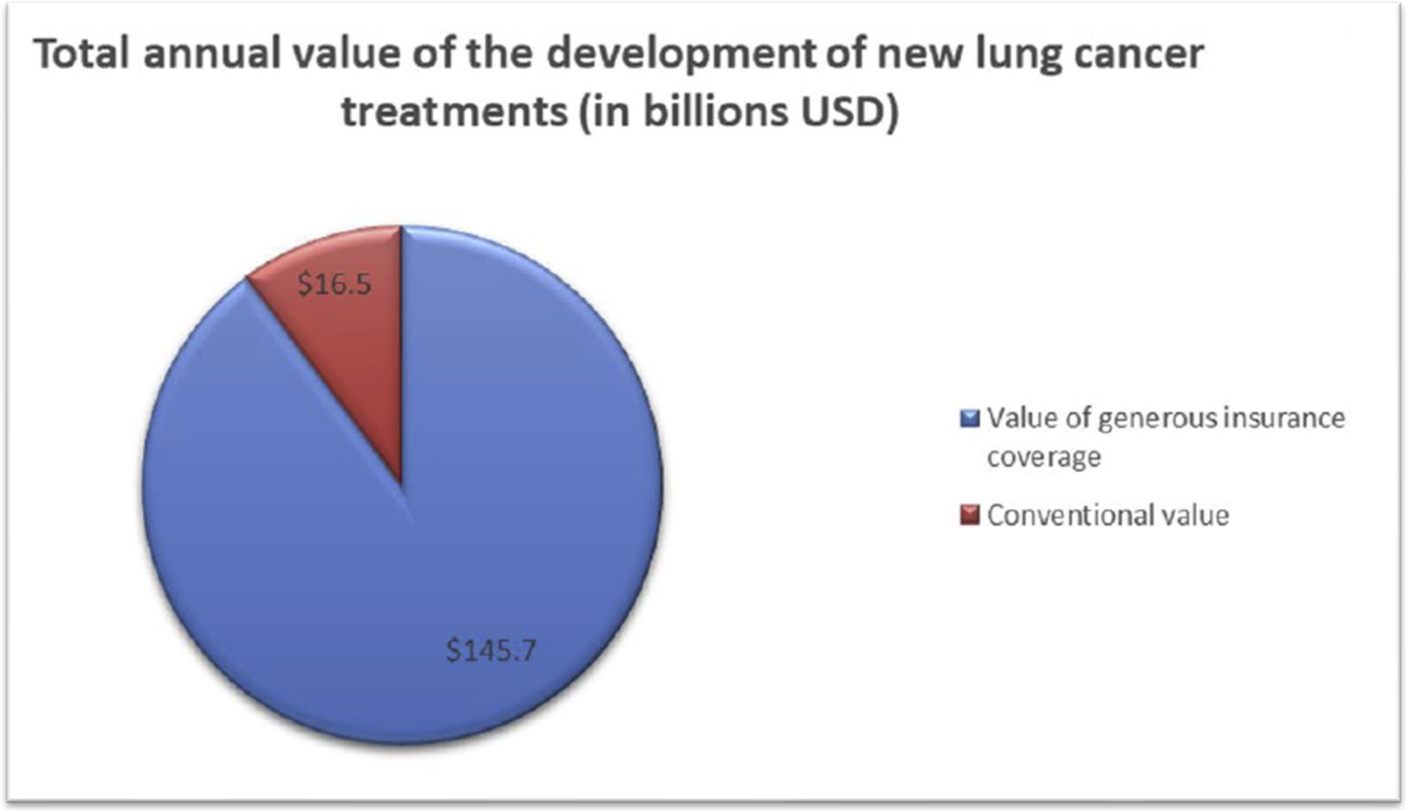
Insurance value: Insurance value can be estimated using stated preference survey methodologies

Figure 1. Sample choice set for survey of adults with no prior or existing cancer.

	Insurance Plan 1 (conventional treatment is available and covered)	Insurance Plan 2 (both conventional and new treatments are available and covered)
Five year survival rate if diagnosed with lung cancer	**4 out of 100** patients receiving conventional treatment will live five years or longer 	**10 out of 100** patients receiving the new treatment will live five years or longer 
Monthly health insurance premium	**None** beyond what you currently pay for health insurance each month	**$50.00** beyond what you currently pay for health insurance each month
Which plan do you prefer?	○	○

Shafrin J. *Value in Health*. 2021 Jun 1;24(6):855-61.

Insurance value: Approach estimates that **~90%** of cancer treatment value comes from non-cancer patients



Relevant when patients have high risk aversion, likely for serious diseases

Shafrin J. *Value in Health*. 2021 Jun 1;24(6):855-61.

Discussion

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