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Improving healthcare decisions

Value Assessment and Reimbursement of Early Treatment for Prevention in Chronic Progressive Diseases: Are Traditional Approaches up to the Task?

ISPOR 2024

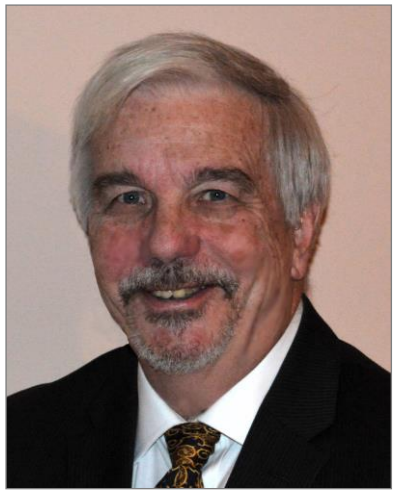
Atlanta, GA, USA

Tuesday, May 7, 2024 | 1:45 PM EST

Welcome

William Herring, PhD

Executive Director
Health Economics
RTI Health Solutions
Research Triangle Park, NC, USA



Charles Phelps, MBA, PhD

University Professor and Provost Emeritus
University of Rochester
Rochester, NY, USA

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Global Health and Population
Harvard T.H. Chan School of Public Health
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Amanda Cole, PhD

Associate Director
Office of Health Economics
London, UK

Overview

- Background and motivation [Herring]
- GRACE for chronic progressive diseases [Phelps]
- Accounting for non-health benefits [Jiao]
- Implications for reimbursement [Cole]
- Discussion / Q & A (15 minutes)

GRACE = Generalized Risk-Adjusted Cost-Effectiveness.

What do we mean by “chronic progressive”?

- **Chronic:** “conditions that last 1 year or more and require ongoing medical attention or limit activities of daily living or both”[1]
- **Progressive:** conditions that get “worse over time, resulting in a general decline in health or function”[2]

Prominent, burdensome conditions

e.g., heart disease, chronic lung disease, diabetes, Alzheimer’s disease

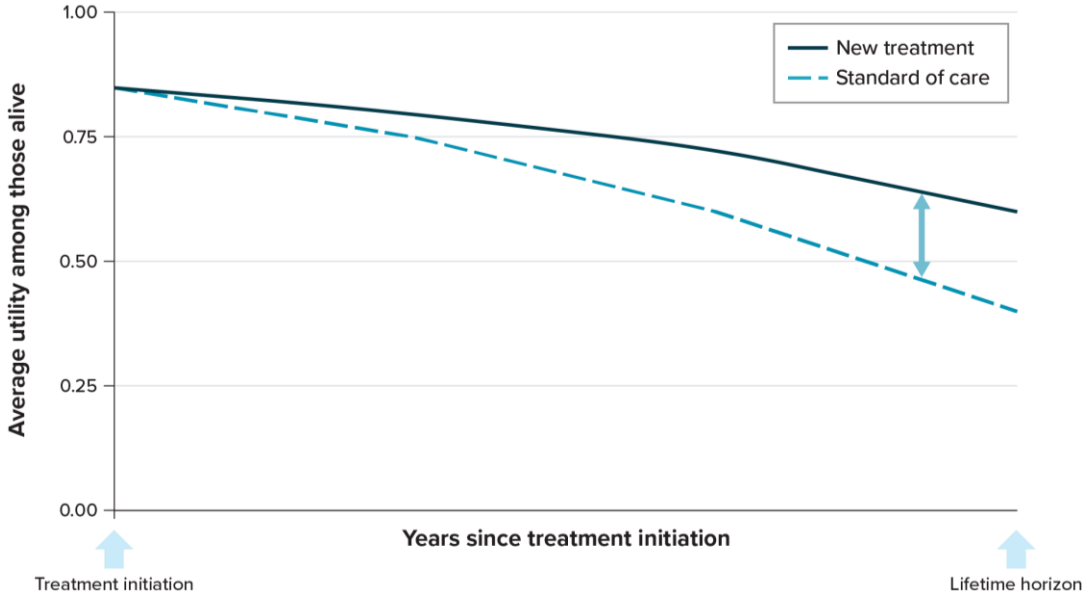
Rare, often genetic, conditions

e.g., amyotrophic lateral sclerosis, sickle cell disease, cystic fibrosis

[1] US Centers for Disease Control and Prevention. https://www.cdc.gov/chronicdisease/about/index.htm. 17 Apr 2024. [2] VeryWellHealth. https://www.verywellhealth.com/what-is-a-progressive-disorder-2564690. 17 Apr 2024.

Why does the "progressive" piece matter?

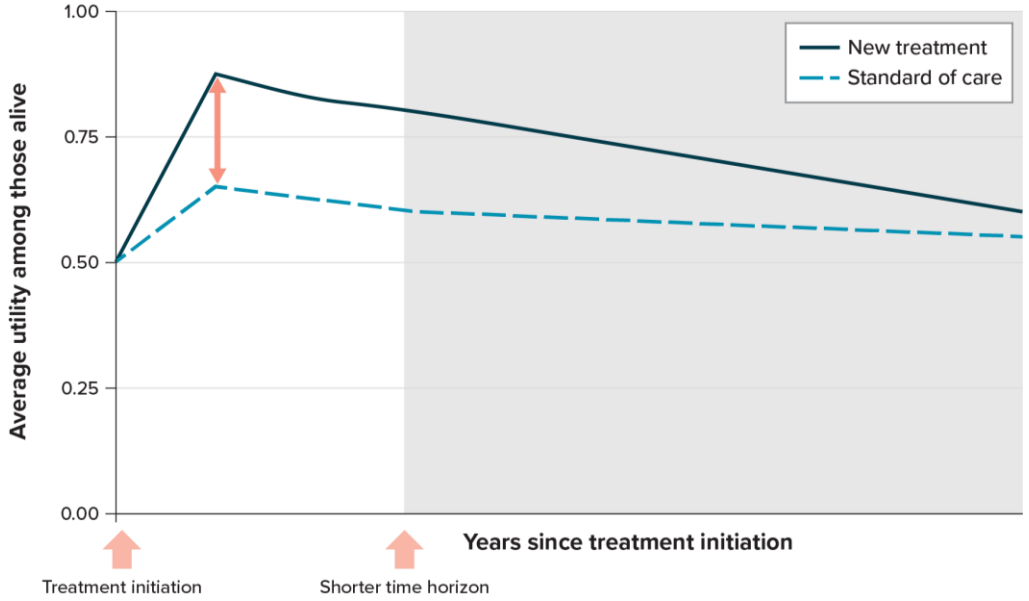
Early treatment to delay or prevent progression



- Natural history of disease progression
- Heterogeneity in the risk of getting advanced disease
- Long-term treatment effect extrapolation and uncertainty
- Treatment costs incurred before benefit is accrued
- Discontinuation and waning have big impacts on cost-effectiveness

vs.

Treatment of symptoms to achieve response



- Limited reliance on natural history
- Patients already have advanced, symptomatic disease
- Heterogeneity in response to treatment
- Treatment costs and benefits accrued at same time
- Discontinuation and waning (loss of response) have less impact on cost-effectiveness

Source: Herring et al. ISPOR 2022 Conference. May 2022.

There is a recurring theme here!

- Alzheimer's disease
- One-time curative gene therapies
- And many more, e.g.

  Parkinson's disease
  Cystic fibrosis
  Nonalcoholic steatohepatitis
  Other rare diseases
  …


EXPERT REVIEW OF PHARMACOECONOMICS & OUTCOMES RESEARCH
2020, VOL. 20, NO. 6, 563–570
https://doi.org/10.1080/14737167.2020.1822738

Taylor & Francis
Taylor & Francis Group

REVIEW

Check for updates

Challenges in demonstrating the value of disease-modifying therapies for Alzheimer's disease

Anders Gustavsson[a], Peter Pemberton-Ross[b], Melissa Gomez Montero[c], Mahmoud Hashim[c] and Robin Thompson[b]


Gene therapy may not be as expensive as people think: challenges in assessing the value of single and short-term therapies

Louis P Garrison, Jr, PhD; Boshen Jiao, MPH; and Omar Dabbous, MD

J Manag Care Spec Pharm.
2021;27(5):674-81


PharmacoEconomics (2023) 41:1205–1228
https://doi.org/10.1007/s40273-023-01289-0

SYSTEMATIC

Modelling
An Updat

Judith Dams[1]

JOURNAL OF MEDICAL ECONOMICS
2022, VOL. 25, NO. 1, 783–791
https://doi.org/10.1080/13696998.2022.2077550
Article 0071-FT.R1/2077550

ORIGINAL RESEARCH

Limitation
assessmer
for cystic

Jaime L. Rubi

Systematic Literatur
Economic Models Us
Steatohepatitis: Pote

Pierre Johansen[1] · Daniel Ho

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VALUE IN HEALTH 21 (2018) 515–524

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Available online at www.sciencedirect.com
ScienceDirect

journal homepage: www.elsevier.com/locate/jval

Economic Modeling Considerations for Rare Diseases

Isobel Pearson, DPhil[1], Ben Rothwell, MSc[1], Andrew Olaye, PhD[2], Christopher Knight, MSc[1,]*

Why Standard CEA Can't Handle Progressive Diseases (and how to fix it)

Charles E Phelps, PhD
University of Rochester
Rochester, NY, USA

Scarcity Matters

- To whom is 500 Square Feet more of floor space more valuable?
  - Two-person family in a 1100 SF apartment in Brooklyn
  - Two-person family in a 3500 SF suburban home?
- In which situation would 1 gallon of water be more valuable to you?
  - Sitting beside your swimming pool, sipping lemonade or iced tea?
  - Stranded 20 miles off of the main road in Death Valley, CA (115 degrees F)
- In which condition is an improvement of 0.1 (scale of 0 to 1) in HRQoL?
  - When you have a moderate headache?
  - When you have persistent migraine headaches?
  - When you have persistent major pain from bone cancer?

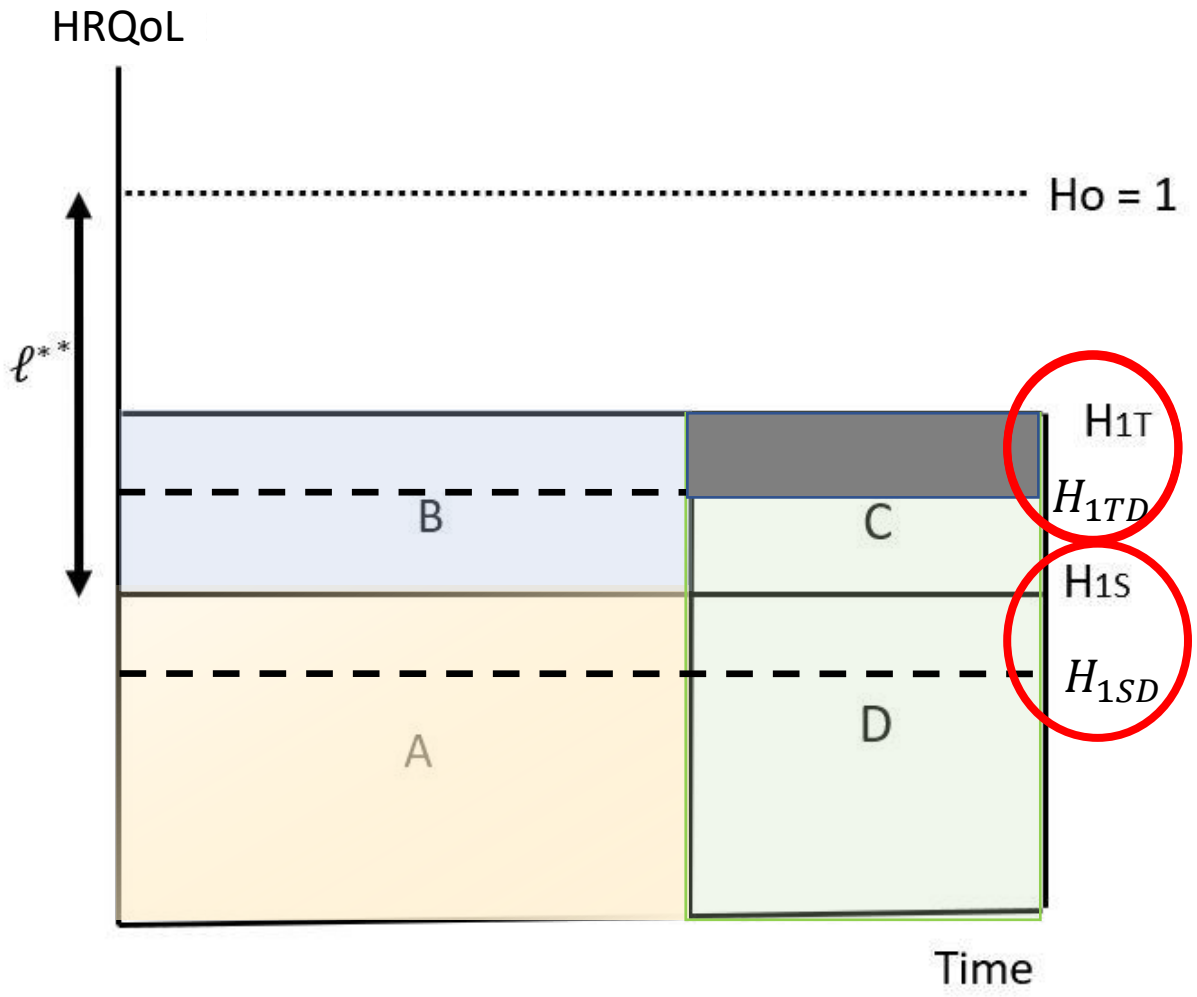
Real People Care about Illness Severity!

- Linley WG, Hughes DA. “Societal views on NICE, cancer drugs fund and value- based pricing criteria for prioritising medicines: A cross- sectional survey of 4118 adults in Great Britain: Societal preferences for the funding of medicines,” *Health Economics* 2013; 22(8): 948– 964.
- Nord E, Pinto JL, Richardson J, Menzel P, Ubel P. “Incorporating societal concerns for fairness in numerical valuations of health programmes,”*Health Economics* 1999; 8(1): 25– 39.
- Shah KK. “Severity of illness and priority setting in healthcare: A review of the literature,” *Health Policy* 2009; 93(2– 3): 77– 84.
- Gu Y, Lancsar E, Ghijben P, Butler JR, Donaldson C. “Attributes and weights in health care priority setting: A systematic review of what counts and to what extent,” *Social Sciences in* Medicine 2015; 146: 41– 52.
- Shiroiwa T, Igarashi A, Fukuda T, Ikeda S. WTP for a QALY and health states: More money for severe health states? *Cost Effectiveness and Resource Allocation* 2013; 11: 22.

Key Issues with Progressive Diseases

- “Progressive” means deteriorating HRQoL (by definition)
- They’re permanent (almost always)
- They reduce life expectancy (often)
- They affect children and young adults (often)
- They create significant caregiver burden (almost always)
- They are rare (often)
- **Standard CEA cannot properly account for any of these issues.**

Gains in LE Don't Count as Much in CEA for Disabled



Gains in LE are shrunk because HRQoL is lower

US Affordable Care Act Bans Use of CEA

- **"The Patient- Centered Outcomes Research Institute … shall not develop or employ a dollars-per- quality adjusted life year (or similar measure that discounts the value of a life because of an individual's disability) as a threshold to establish what type of health care is cost effective or recommended.**

US Affordable Care Act Bans Use of CEA

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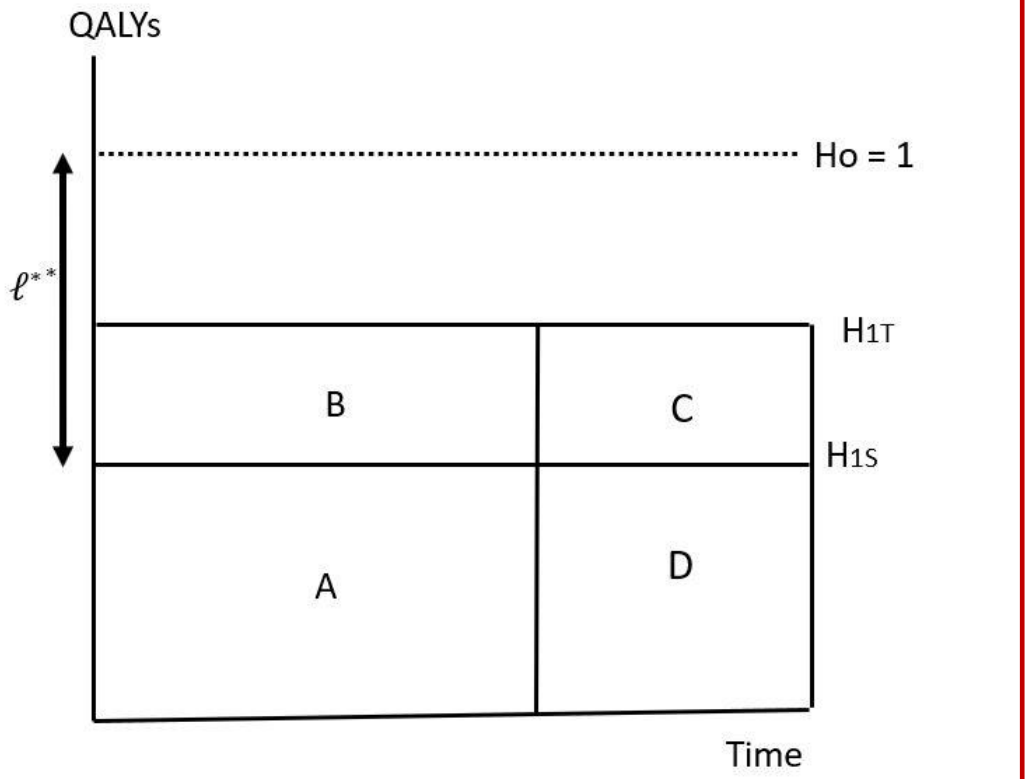
Ad Hoc Severity Adjustments Abound



- AS = Absolute shortfall: total QALYs lost from untreated disease
- PS = Proportional shortfall: relative loss of QALYs from untreated disease

- AS = B + C + D
- PS = AS/(A+B+C+D)

Phelps CE, Lakdawalla DL, "Methods to Adjust Willingness to Pay (WTP) Measures for Severity of Illness," *Value in Health* 2023; 26(7):1003-1010

These Methods Have No Basis in Theory

- The “stairstep” methods violate both horizontal and vertical equity
  - People near the boundaries between stairsteps are nearly the same, but treated very differently
  - People within each “stairstep” are different, but are treated the same
- Both AS and PS combine gains in HRQoL and LE irrationally
- AS gives more value per QALY for larger total gains
  - This is backwards from diminishing marginal utility logic
  - PS shares the same problem
- They both can’t be right, that’s for sure!
  - But the BNHS uses both in the UK

Uses of AS and PS Wander All Over the Map

Table 1B. Severity Adjustment in Norway.

AS	NOK threshold	Multiplier
0-3.9	275 000	1
4-7.9	385 000	1.4
8-11.9	495 000	1.8
12-15.9	605 000	2.2
16-19.9	715 000	2.6
20+	825 000	3

Table 1C. Severity Adjustment in The Netherlands.

PS	€ Threshold	Multiplier
0-0.1	(not covered)	——
1.1-0.4	20000	1
0.41-0.7	50000	2.5
0.71-1	80000	4.0

Table 1A. Severity adjustment in the United Kingdom (NICE).

Multiplier	AS	PS
1	< 12	< 0.85
1.2	12-18	0.85-0.95
1.7	≥ 18	≥ 0.95

"Equal Value of Life Years Gained" (EVLYG) and "Healthy Years in Total" ((HYT) Both Have Problems

- Neither rest on well-articulated micro-economic foundations
- EVLYG omits HRQoL gains for gains in LE for
- EVLYG produces bizarre results with standard social welfare functions
- Both are inconsistent when the standard of care (SoC) changes
- Unbounded average value of survival gains in some cases
- Non-convex survival preferences

Paulden, Sampson, O'Mahoney et al, "Logical Inconsistencies in Healthy Years in Total and Equal Value of Life Years Gained," *Value in Health* 2024; 27(3):356-366

Lakdawalla and Doctor, "A principled approach to non-discrimination in cost-effectiveness," *European Journal of Health Economics* 2024; /doi.org/10.1007/s10198-023-01659-7

GRACE fixes these problems

- “Progressive” means deteriorating HRQoL (by definition)
  - GRACE provides a proper method to adjust for HRQoL
- They’re permanent (almost always)
  - GRACE provides a proper method to account for disability
- They reduce life expectancy (often)
  - GRACE properly values LE gains
- They affect children and young adults (often)
  - GRACE properly accounts for large potential losses in LE
- They create significant caregiver burden (almost always)
  - GRACE proves a natural pathway to account for caregiver burden
- They are rare (often)
  - GRACE, combined with appropriate social welfare function, fixes this too.

Scarcity adds five new parameters to the model

- Preexisting Disability multiplier "D"
  - D =1 with no disability, rises exponentially with increasing disability
- Acute illness severity multiplier "R"
  - R = 1 with no acute illness, rises exponentially with acute illness severity
- "Exchange rate" ρ between HRQoL and LE that accounts for scarcity
  - Replaces the fixed exchange rate $\overline{H}$ in standard CEA
  - $\overline{H}$ is the proportional loss in health in disabled state; ρ is the proportional loss in utility
- Multiplier ω_H to account for diminishing returns to health
  - $0 < \omega_H < 1$ in GRACE and $\omega_H = 1$ in standard CEA
  - Applied equally to disabled and non-disabled, and to all degrees of HRQoL loss
- Accounts for uncertain treatment outcomes using risk aversion measures (ϵ)
  - Treatment value rises if it reduces uncertainty
  - Not relevant to progressive disease issue, so we can ignore it hereafter

One-Period Total Value of a Medical Intervention

$$TVMI_{CEA} = K\{\mu_p \bar{H} + \mu_B\} \text{ (="QALYs")}$$

$$TVMI_{GRACE} = KD\{\mu_p \rho + \omega_H R \mu_B\}$$

- K *is WTP for one year at* $H_0 = 1$
- μ_p *is for LE*
- μ_B *is for HRQoL*
- $\bar{H}$ is proportional loss in HRQoL due to disability
  - $0 \leq \bar{H} \leq 1$

- D *is disability adjustment*
- R *is acute illness severity adjustment*
- ρ is the proportional loss in *utility* of HRQoL
  - $0 \leq \rho \leq 1$
- ω_H *accounts for diminishing returns to* H
- $\rho D \geq 1$

GRACE DOES NOT DISCRIMINATE AGAINST DISABLED PEOPLE

- GRACE, when configured properly, does not discriminate against disabled people.

$$TVMI_{GRACE} = KD\{\mu_p\rho + \omega_H R\mu_B\}$$

- Gains in HRQoL, μ_B, are multiplied by DR, where $D \geq 1$ and $R \geq 1$
  - Both *D* and *R* rise exponentially with the degree of disability
- Gains in LE, μ_p are multiplied by $D\rho$ where $D\rho \geq 1$ when properly configured
  - Method 1: Combine illness and disability gains multiplicatively, not additively
    - $d^* = \%\ loss\ due\ to\ disability;\ \ \ell^* = \%\ loss\ due\ to\ acute\ illness; H_S = H_0(1-d^*)(1-\ell^*)$
  - Method 2: Use Constant Relative Risk Aversion (CRRA) utility in health
    - A standard "workhorse" in health economics and economics in general
  - Method 3 (in progress): Use Expo-Power (EP) utility
    - Highly flexible, accommodates many forms of risk preferences

The Multi Period Model (Apologies for the Math, It's Necessary for Progressive Diseases)

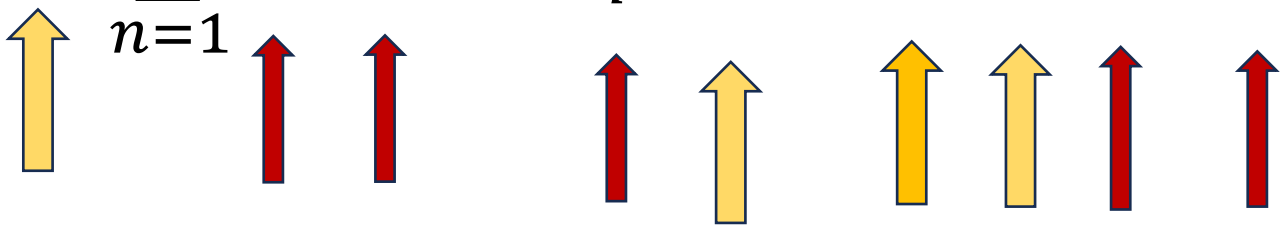
$$\beta = \frac{1}{1+r} \qquad \beta^n = \left[\frac{1}{1+r}\right]^n$$

p_n =period to period survival probability

$$\Pi_n = \prod_{n=1}^{n} p_n$$

$$\mathrm{TVMI}_{\mathrm{CEA}} = K \sum_{n=1}^{N} \beta^n \Pi_{n-1}\left\{\left[\mu_{pn}\bar{H} + p_n\mu_{Bn}\right]\right\} \quad (= \text{“QALYs”})$$

$$\mathrm{TVMI}_{\mathrm{GRACE}} = KD \sum_{n=1}^{N} \beta^n \Pi_{n-1}\{[\mu_{pn}\rho_n + \omega_H R_n p_n \mu_{Bn}]\}$$

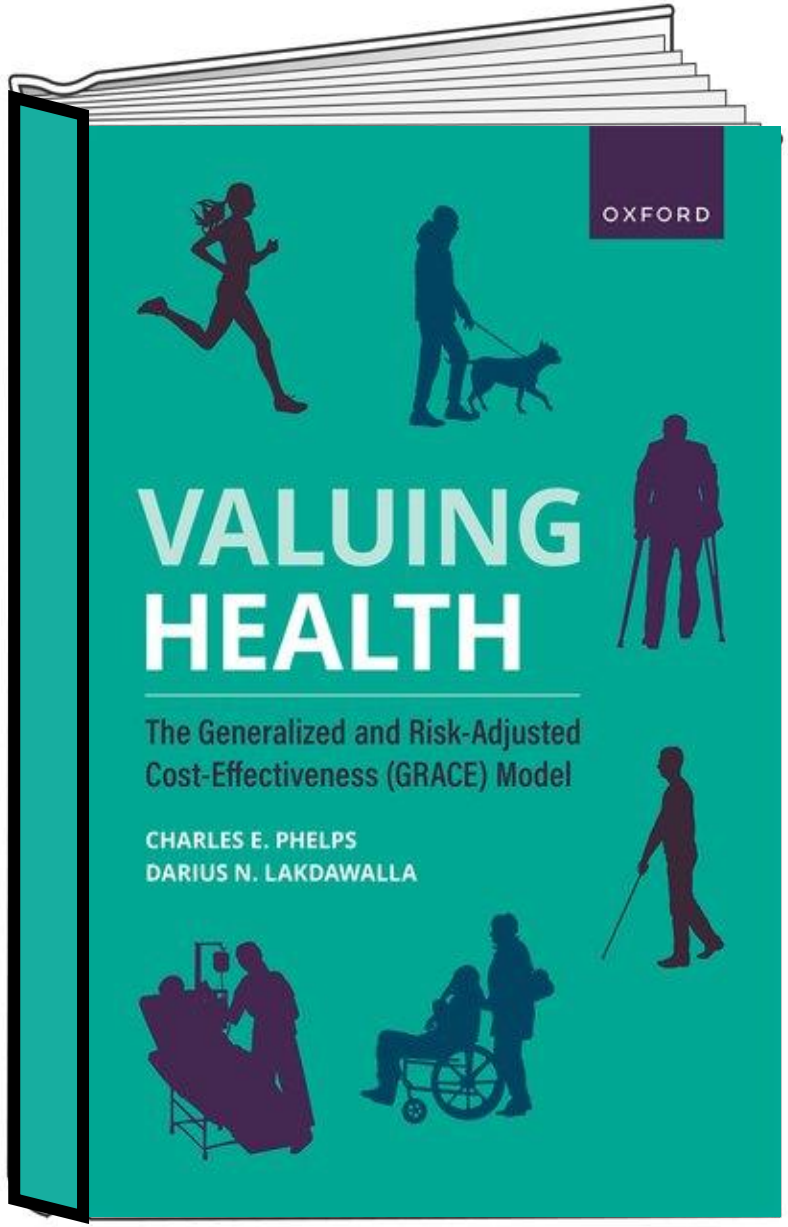


Take Home Messages

- Standard CEA ignores severity, so “progressive” diseases are treated the same at all levels of progression. **THIS IS WRONG!**
- GRACE provides clear and theory-based methods to adjust for severity.
- Most of the values in the multi-period model are measured anyway in any competent RCT or similar multi-period analysis.
- The only “new” things needed are measures of attitudes towards risk
  - The function and parameters for $W(H)$ that replace H
  - Utility of health replaces health itself
- GRACE is legal within US Affordable Care Act restrictions on CEA

SHAMELESS PROMOTION

(February 2024)



Thanks For Your Attention

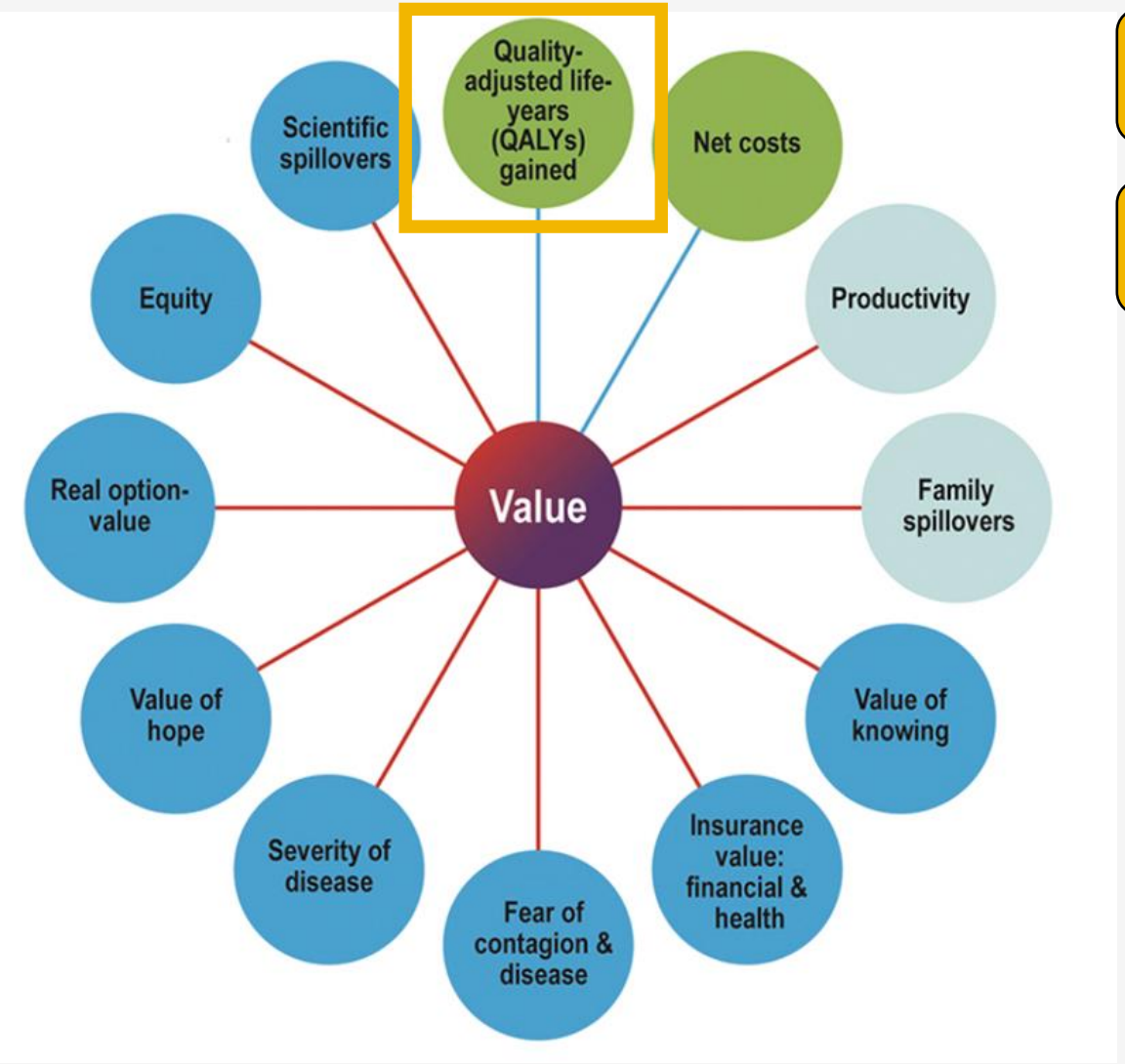
How GRACE and CEA define a treatment's value

- Define improvement in survival probability as μ_p
- Define improvement in HRQoL as μ_B ("benefit")
- Define baseline health as $H_0 = 1$
- Define relative acute illness health loss as ℓ^*, so $H_S = H_0(1 - \ell^*)$
- Define relative disability health loss as d^*, so $H_D = H_0(1 - d^*)$
- Define average post-treatment health as $\overline{H}$

Incorporating **Non-Health Benefits** into Value Assessment of Early Treatment in Chronic Progressive Diseases

Boshen Jiao, PhD, MPH
Harvard University

Societal value of early treatment



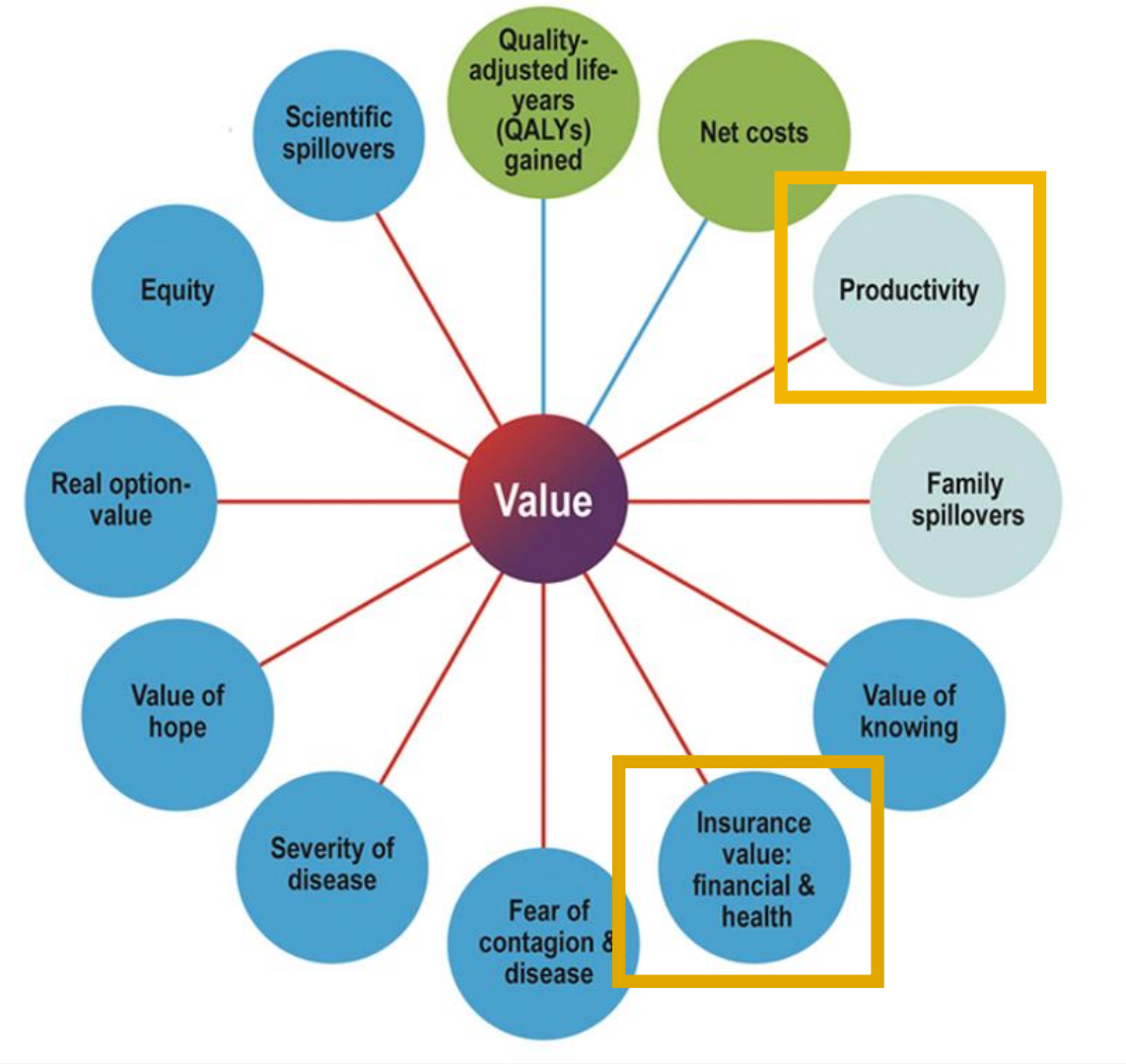
Survival

Quality of life

Health benefits

Source: Lakdawalla et al. 2018, *Value in Health*

Societal value of early treatment



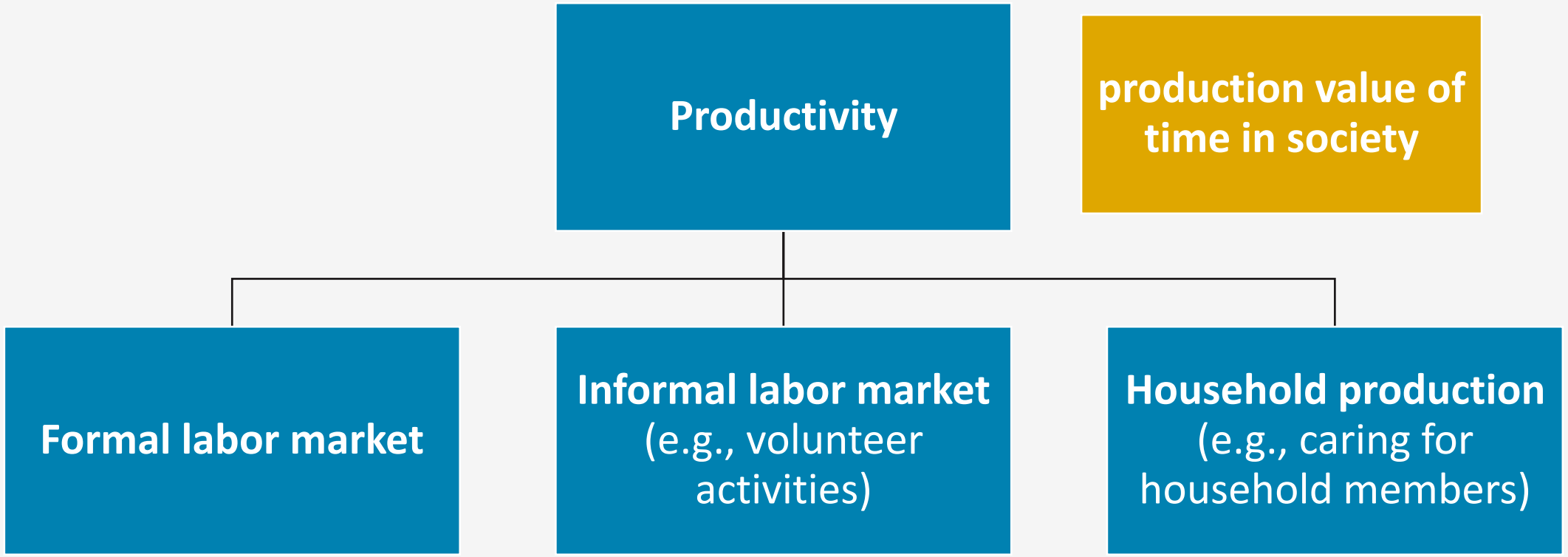
Productivity

Financial risk protection

Non-health benefits

Source: Lakdawalla et al. 2018, *Value in Health*

Productivity

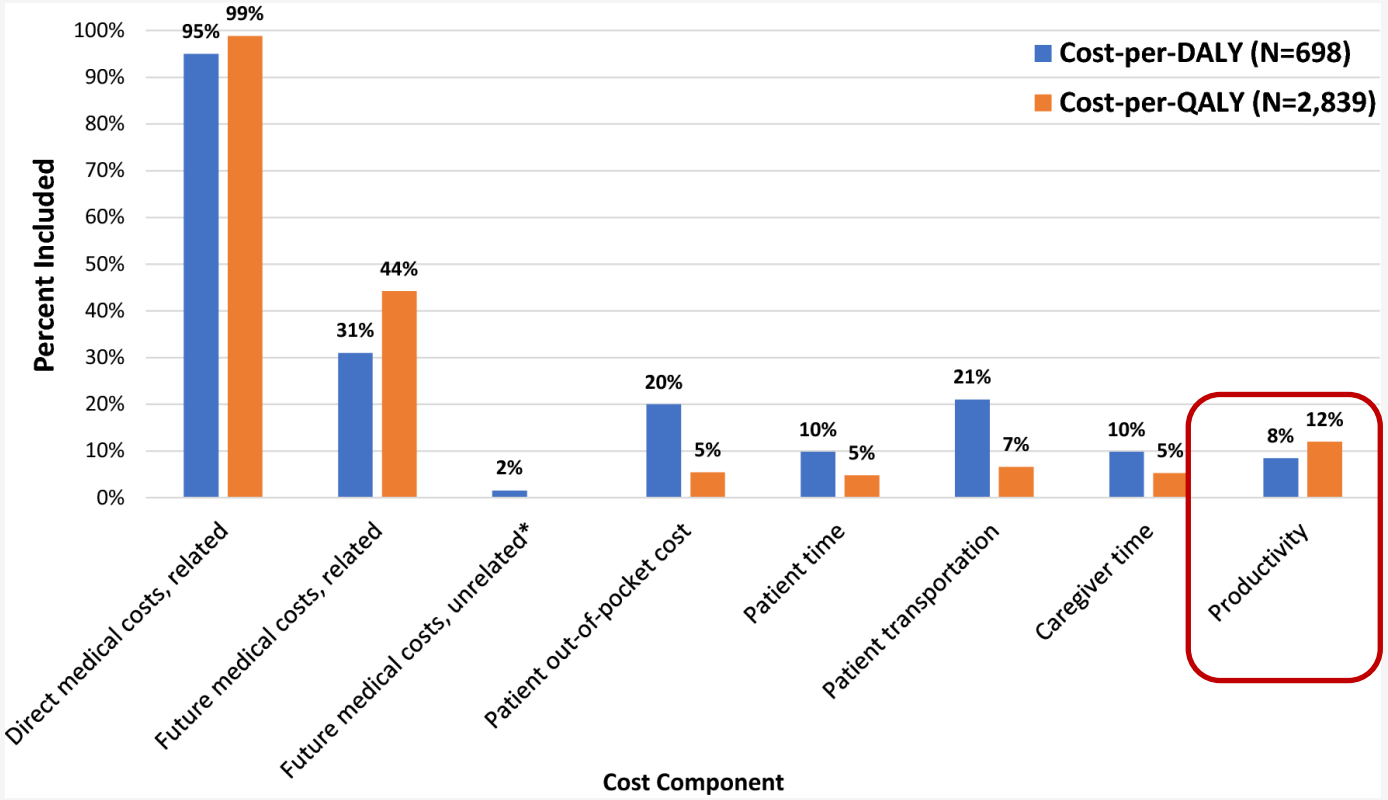


Early treatment for prevention

Early treatment for prevention

Data challenge

- Ideal approach: measure productivity prospectively in trials
- However, such systematic data usually not available
  - ➢Inconsistent inclusion of productivity in published CEAs



~10% published CEAs include productivity

Source: Kim et al. 2020. *Pharmacoeconomics*

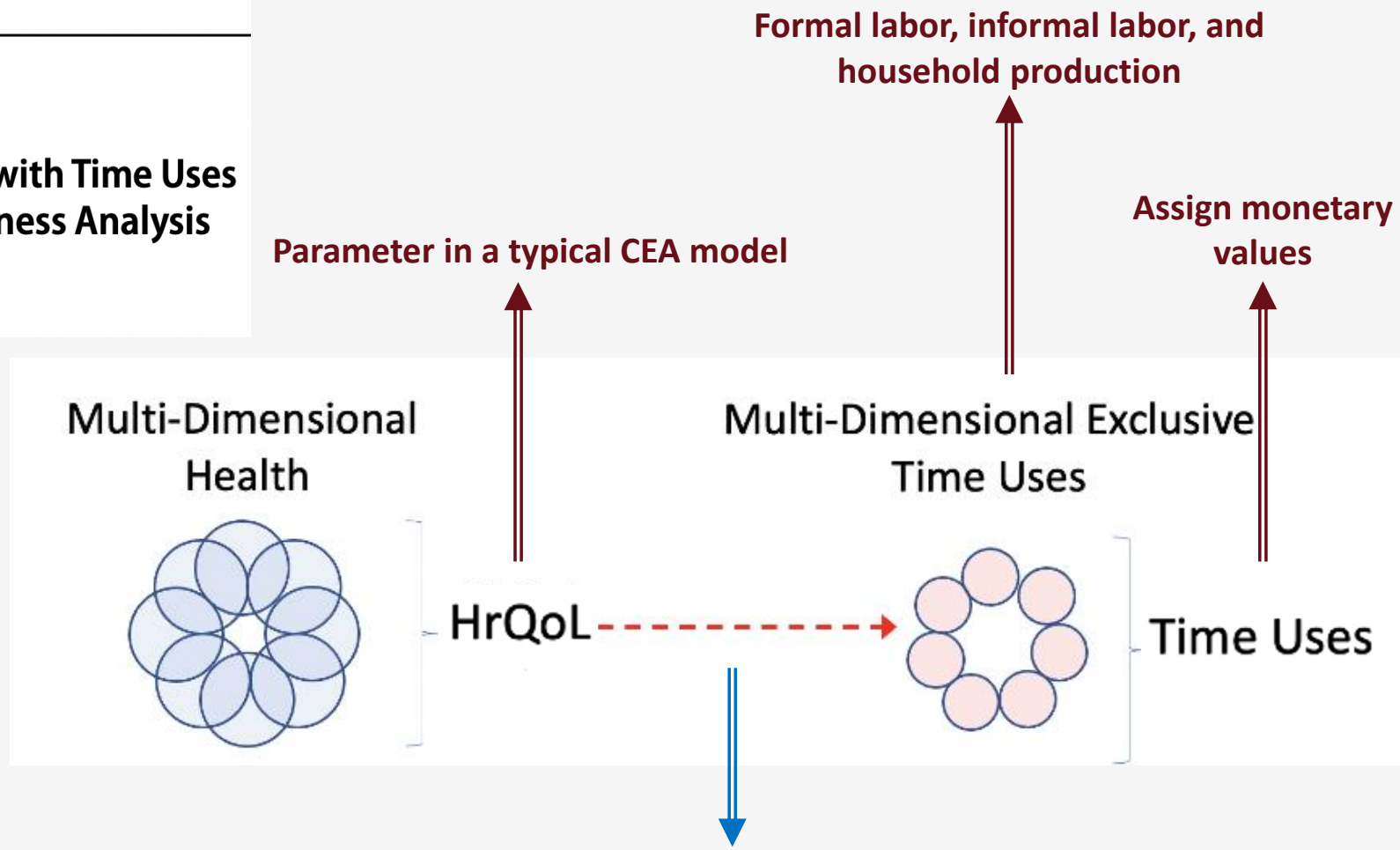
New method

PharmacoEconomics (2023) 41:1065–1077
https://doi.org/10.1007/s40273-023-01246-x

ORIGINAL RESEARCH ARTICLE

Associating Health-Related Quality-of-Life Score with Time Uses to Inform Productivity Measures in Cost-Effectiveness Analysis

Boshen Jiao[1] · Anirban Basu[1]



Case study: Gene therapy for sickle cell disease

Annals of Internal Medicine

ORIGINAL RESEARCH

Gene Therapy Versus Common Care for Eligible Individuals With Sickle Cell Disease in the United States

A Cost-Effectiveness Analysis

Anirban Basu, PhD; Aaron N. Winn, PhD; Kate M. Johnson, PhD; Boshen Jiao, PhD, MPH; Beth Devine, PhD, PharmD, MBA; Jane S. Hankins, MD, MS; Staci D. Arnold, MD, MBA, MPH; M.A. Bender, MD; and Scott D. Ramsey, MD, PhD

- Incremental medical costs: $2,299,000
- **ICER: $193,000/QALY**

Health care sector perspective

Incremental non-medical costs

- Productivity: −$1,248,000
- Patient time : −$29,000
- Caregiver time : −$19,000
- Consumption: $496,000

- Incremental costs: $1,499,000
- **ICER: $126,000/QALY**

Societal perspective

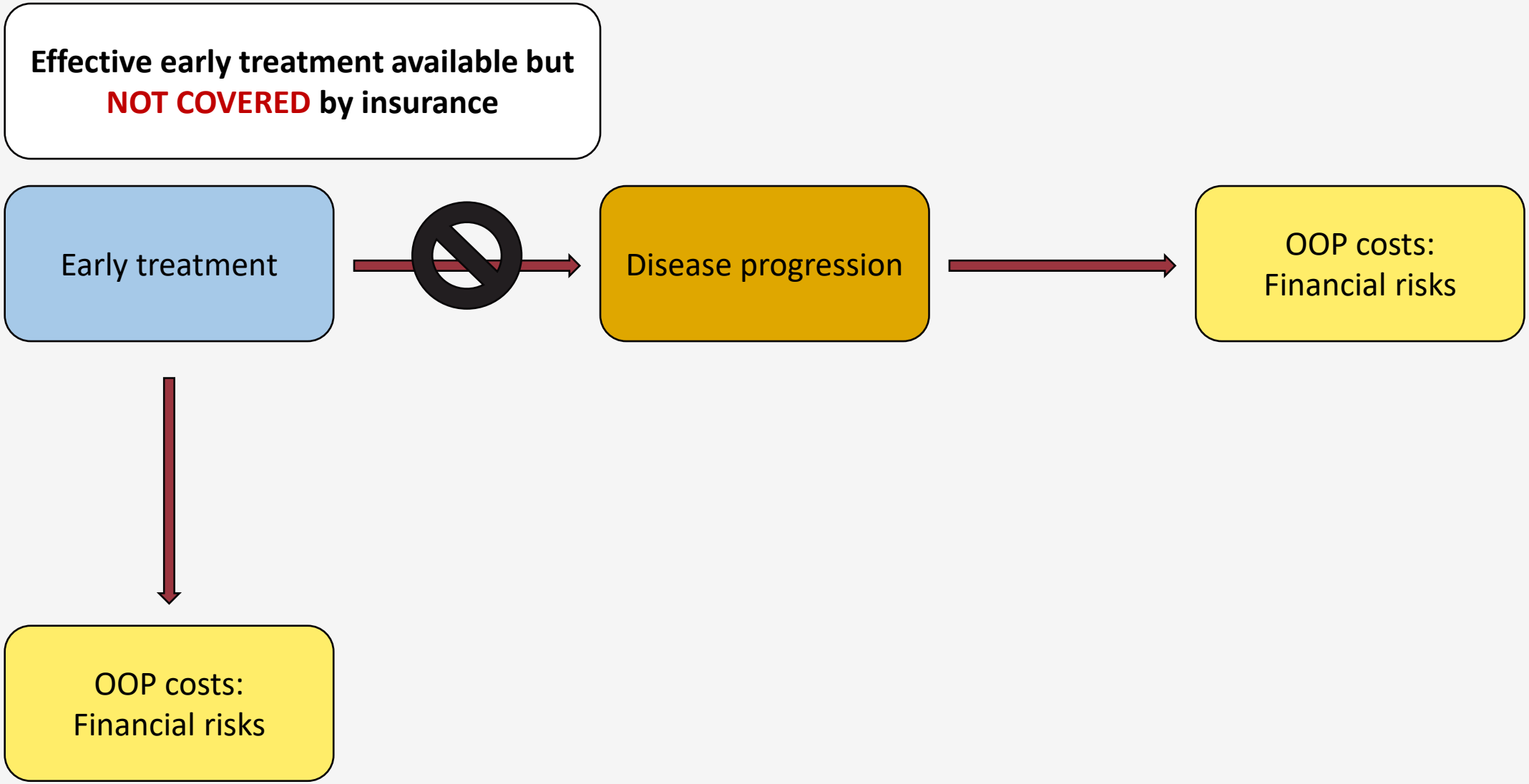
Apply the algorithm to estimate formal labor, informal labor, household production, and time seeking care for persons aged ≥15 years

Financial risk protection

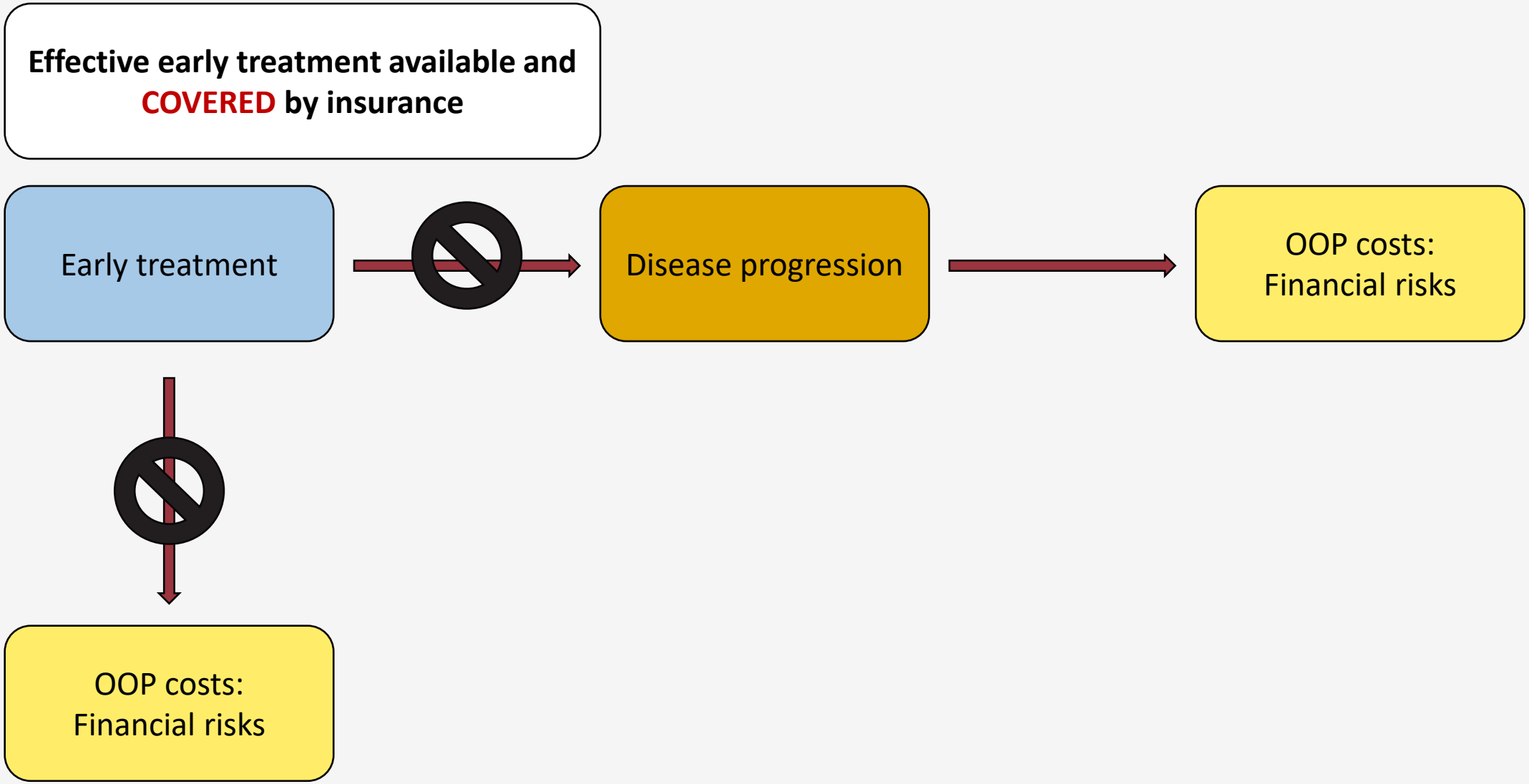
- Financial risk protection (FRP): protecting patients and their caregivers from **financial difficulties** associated with paying for **out-of-pocket (OOP)** expenditures

Early treatment for prevention

Early treatment for prevention



Early treatment for prevention



Equity implications

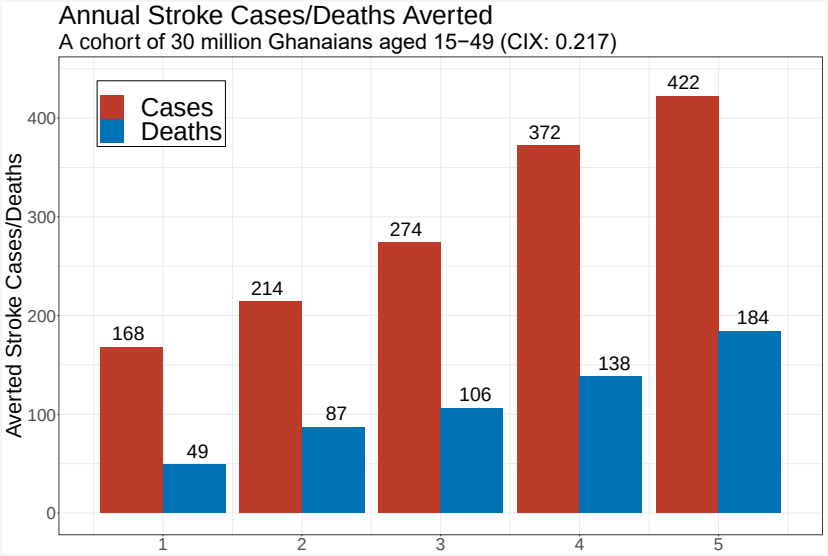
Applied Health Economics and Health Policy
https://doi.org/10.1007/s40258-024-00871-7

ORIGINAL RESEARCH ARTICLE

Health Interventions May Have Divergent Impacts on Health and Economic Equity: A Case Study of the Community-Based Hypertension Improvement Project in Ghana

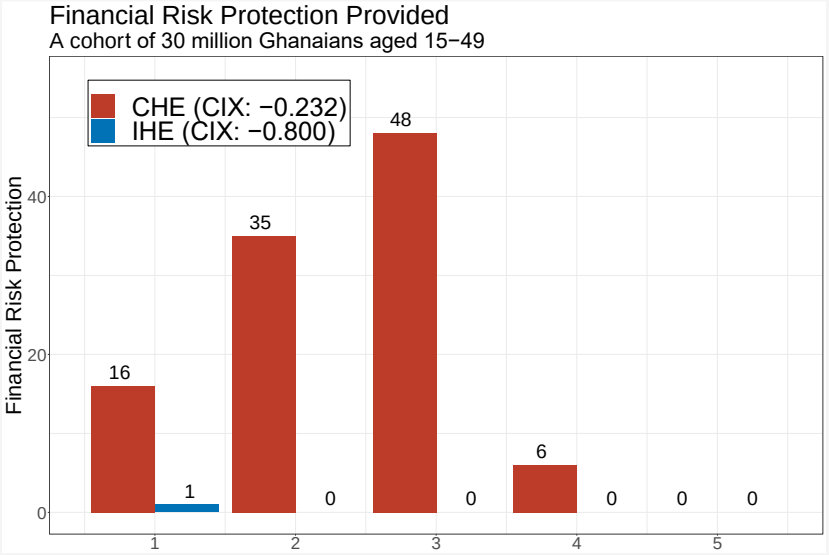
Yizhi Liang[1] · Yuqian Lin[1] · Boshen Jiao[1]

Health equity



Wealth quintile: Poorest → Richest

Economic equity



Assessment approach

Extended cost-effectiveness analysis (ECEA)

Verguet et al. 2016, *PharmacoEconomics*

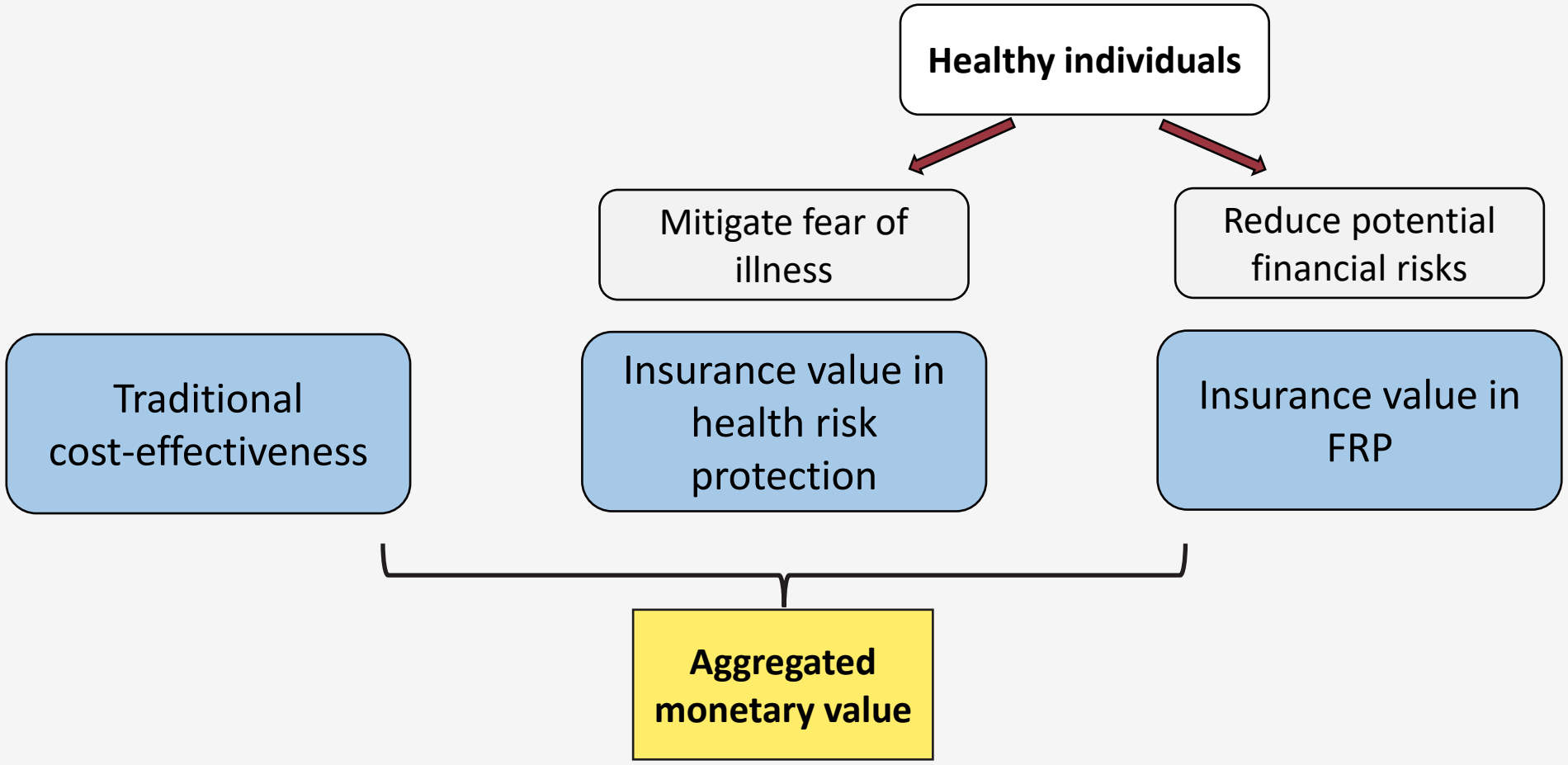
Poorest	Poorer	Middle	Richer	Richest
$/Health gained	$/Health gained	$/Health gained	$/Health gained	$/Health gained
$/FRP gained	$/FRP gained	$/FRP gained	$/FRP gained	$/FRP gained

Disaggregated analysis

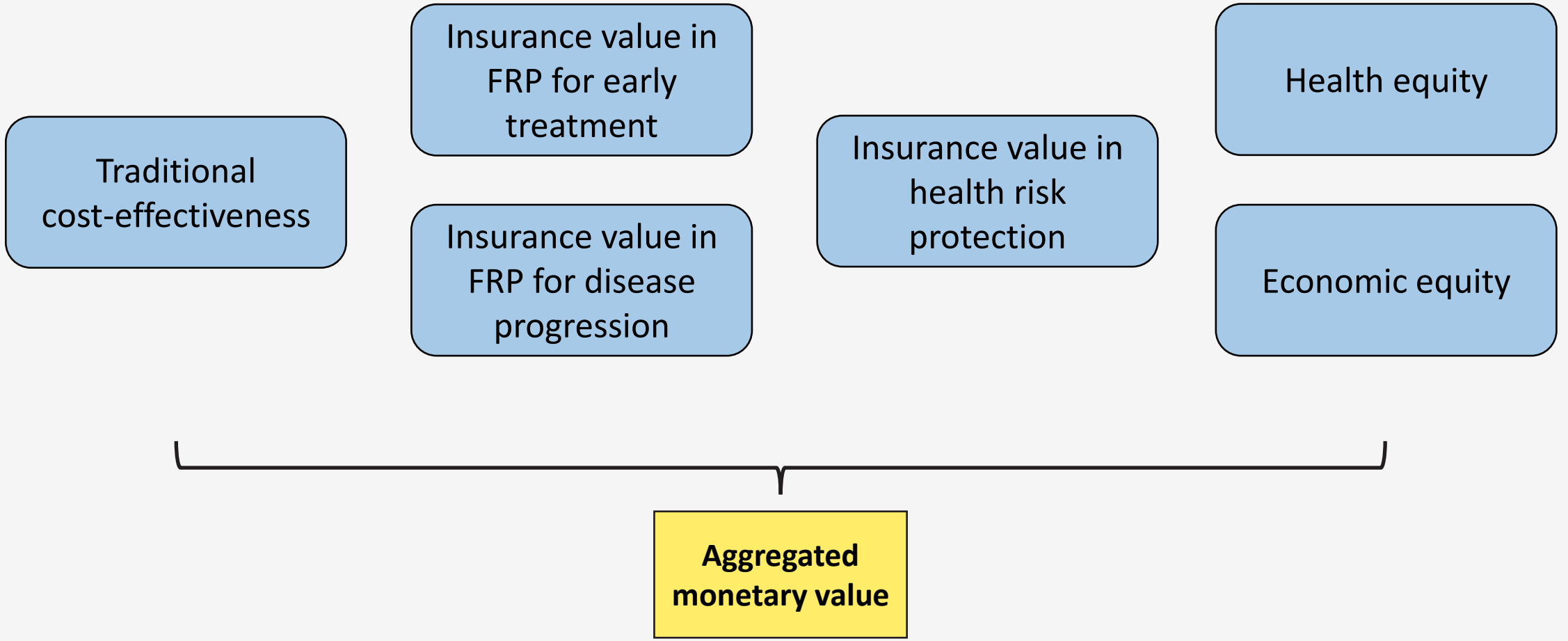
Assessment approach

Insurance value of medical intervention

Lakdawalla et al. 2017, *Journal of Public Economics*



New method



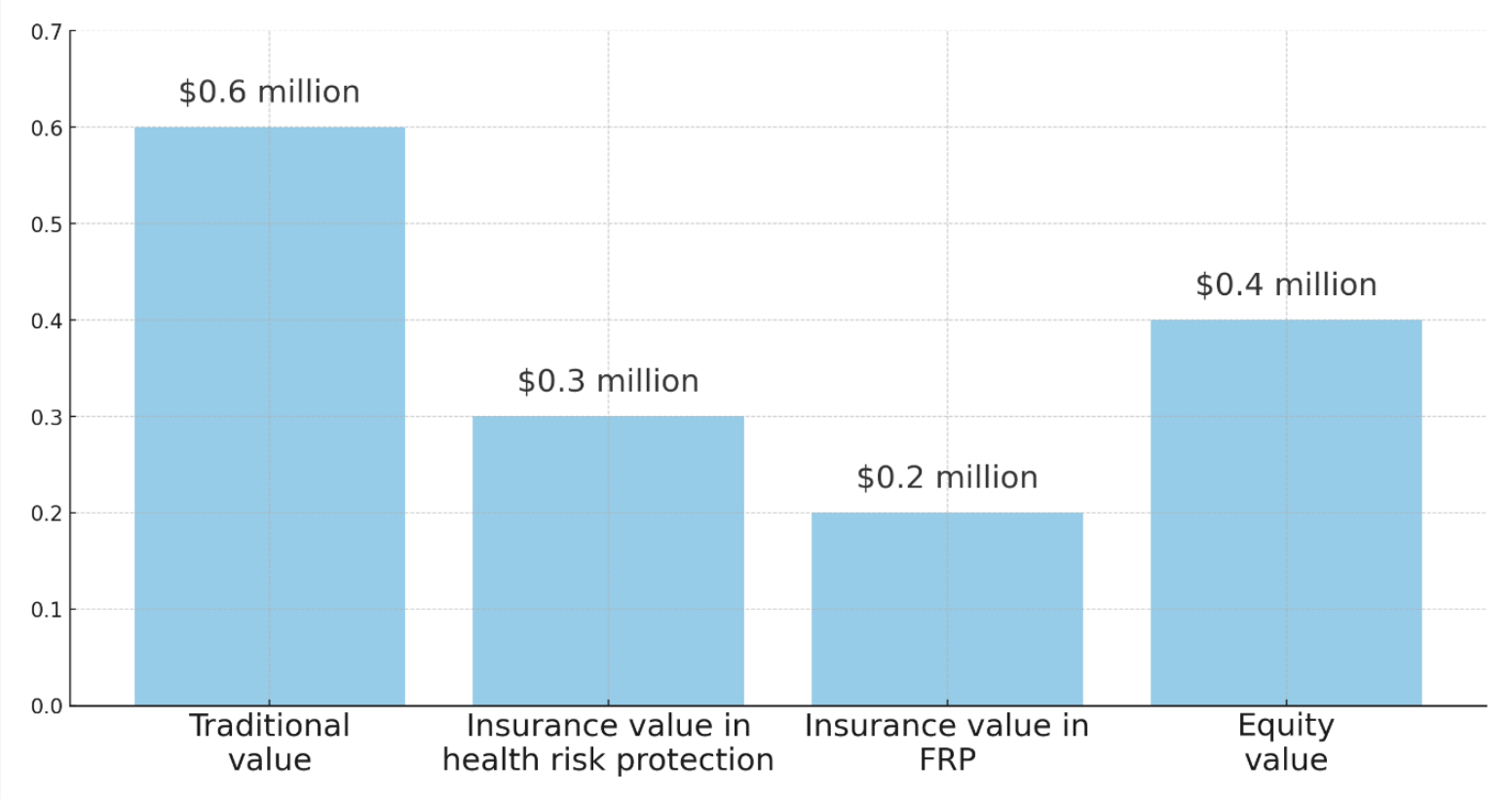
Jiao et al. 2024, *under preparation*

Illustrative case study

- Setting: average income of $2,000 with inequality
- Chronic progressive disease incidence: 1 per 1,000 impacts poorer populations more
- Disease progression costs: $500 OOP
- Early treatment yields 0.5 QALY gained
- Cost of early treatment: $1,000
- Assumes risk and inequality aversion

Total value: $1.4 million

Early treatment, fully insured, for a population of 1 million



Take-aways

- Early effective treatment offers **more than health benefits**
- The **non-health benefits** of early treatment should be integrated into societal-perspective **value assessment**
  - Excluding these benefits could significantly underestimate the true value
- **New methods** have emerged to integrate these benefits into value assessments

Reimbursement of early treatments for chronic progressive diseases

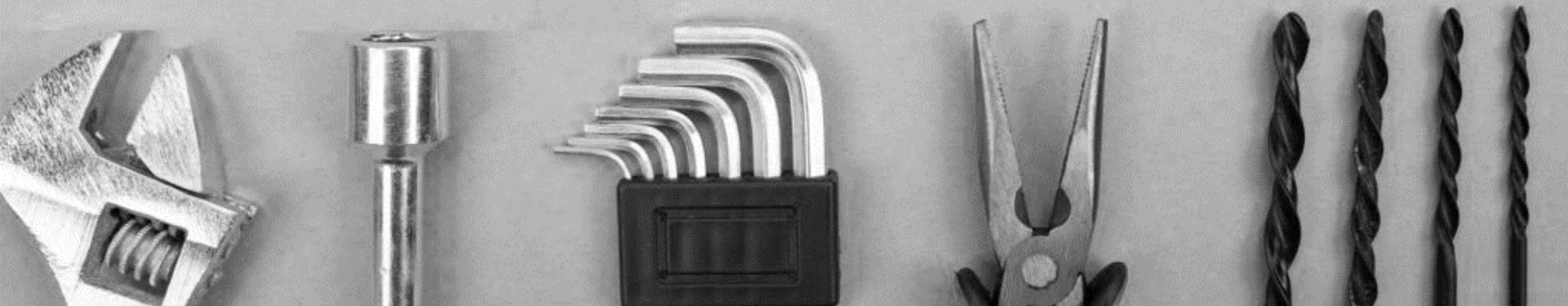
Are traditional approaches up to the task?

ISPOR International, Atlanta, May 7th 2024

Prof Amanda Cole, PhD.

Associate Director – The Office of Health Economics

Honorary Professor of Practice – University College London



Outline

1. Early treatment for chronic progressive diseases:
   Two case examples

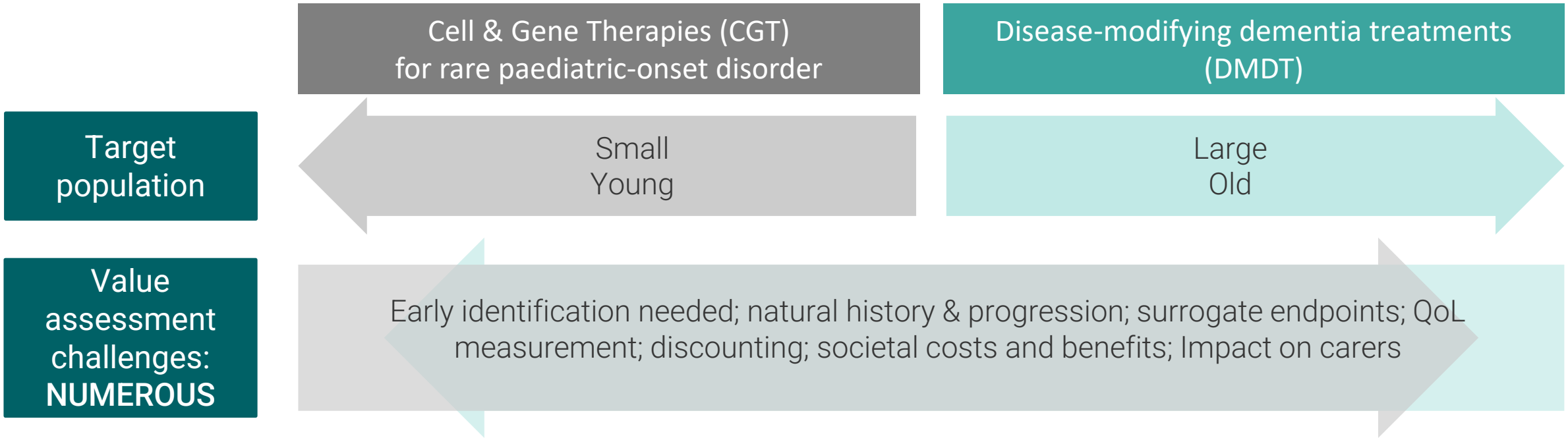
2. Are traditional reimbursement models up to the task?
   Why we need to evolve the way we pay

3. Reimbursement model options

4. Key facilitators

5. Conclusion

Early Treatment for Chronic Progressive disease



Early Treatment for Chronic Progressive disease

	Cell & Gene Therapies (CGT) for rare paediatric-onset disorder	Disease-modifying dementia treatments (DMDT)
Target population	Small Young	Large Old
Value assessment challenges: **NUMEROUS**	Early identification needed; natural history & progression; surrogate endpoints; QoL measurement; discounting; societal costs and benefits; Impact on carers	

*Are traditional **value assessment** approaches up to task?*

NICE Highly Specialised Technologies (HST) guidance for very rare conditions:

- ✓ Higher cost-effectiveness threshold (£100k/QALY)
- ✓ Acceptance in some circumstances of non-reference-case discount rate for benefits (1.5%)
- ✓ Acknowledgement of evidence challenges

> **NICE gets ready to assess new dementia treatments.**
>
> NICE's methods and processes for evaluating new treatments for use in the NHS are appropriate for the new class of Alzheimer's drugs but key issues need to be considered, a new report has found.

Early Treatment for Chronic Progressive disease

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Potential value: **HIGH**	➢ Modify or eliminate disease ➢ Potential for lifetime benefits	
Uncertainty: **HIGH**	➢ High unmet need → accelerated approval ➢ Limited long-term evidence of effectiveness & heterogeneity of treatment response	
Stakes: **HIGH**	➢ Budget impact for payers ➢ **Urgency** for patients	

*Are traditional **reimbursement** approaches up to task?*

Are traditional reimbursement models up to the task? No

OHE

- Optimal pricing must balance *affordability*, *access* and *innovation*

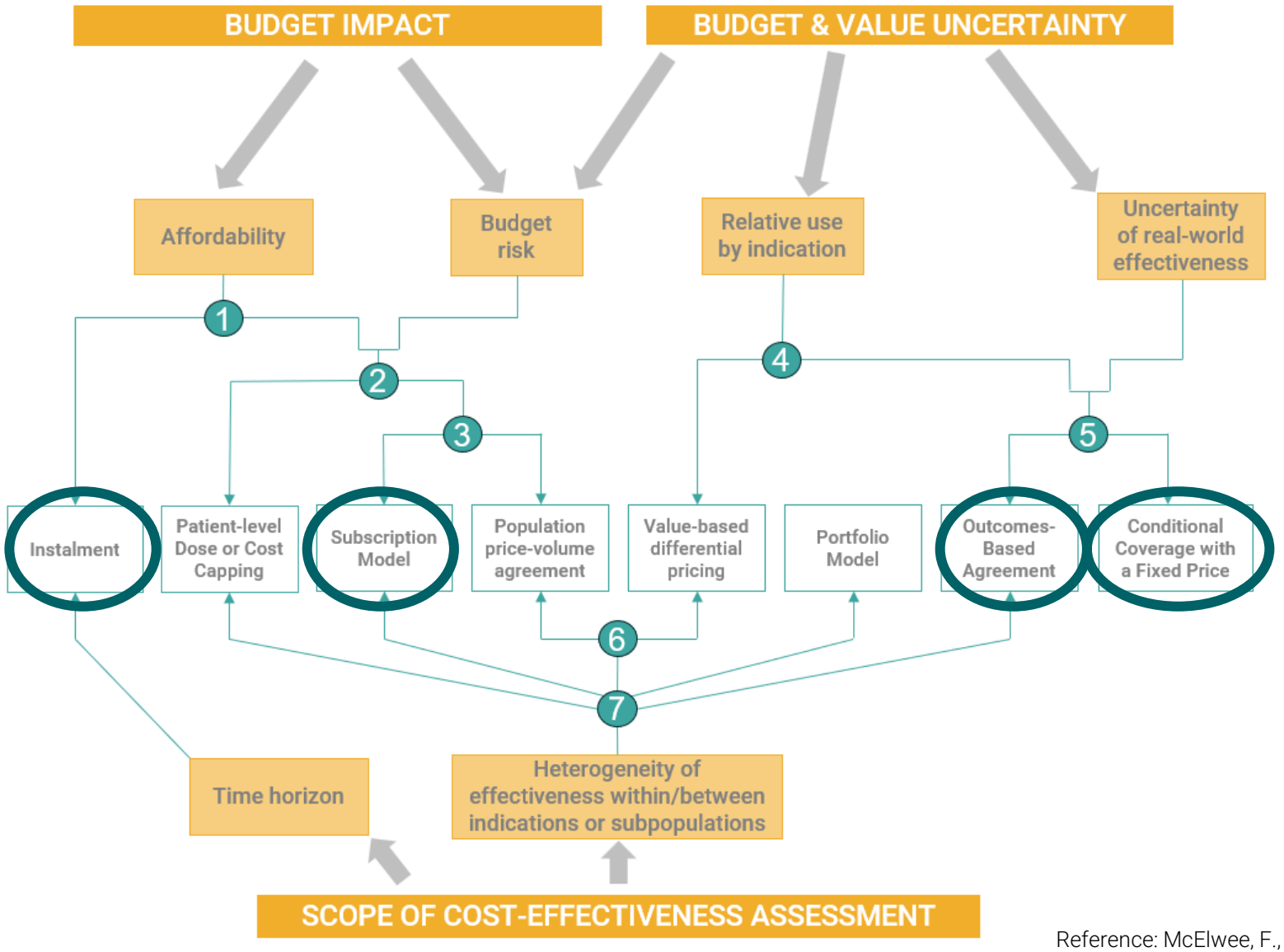
RISK OF TRADITIONAL APPROACH

  - **Payers**: No confirmatory evidence that new treatment will represent a cost-effective use of resources. (… Risk of doing more harm than good to reimburse. Also, I can't afford it).
  - **Patients**: No access to a potentially life-changing treatment. (…Disease progression, and maybe I miss my chance).
  - **Industry**: I run a high risk of no return on investment. (… So, I'll invest in something safer).

- Alternative approaches are needed, but we need to be clear what we're solving for:

CAN ALTERNATIVE MODELS HELP?

  - **Value uncertainty**: payment models to manage and share risk to facilitate *timely patient access* while the *evidence evolves*.
    - Coverage with evidence development; Outcome-based agreements
  - **Affordability or budget risk**: payment models to address short-term budget impact or mitigate budget risk.
    - Instalments; Subscription agreements



Contracting Question		How to proceed in the decision diagram
1.	Is the issue how costs are incurred over time?	Yes / No → 1
2.	Should cost containment apply at the individual- or population-level?	Individual-level / Population-level → 2
3.	What are payer and manufacturer preferences for trading-off Cost-Effectiveness (CE) risk & Budget Risk (BR)?	Prefer (relatively) high CE-risk, low BR / Prefers (relatively) low CE-risk, high BR → 3
4.	Does effectiveness differ between discrete and well-defined subgroups?	Yes / No → 4
5.	Should the price be adjusted retrospectively in light of new evidence?	Prefers (relatively) low CE-risk, high BR / Yes / No / Prefers (relatively) high CE-risk, low BR → 5
6.	What is the appropriate model in the context of other contracting challenges?	6 — Model choice is driven by other factors such as uncertainty and implementation costs.
7.	What is the appropriate type of price discrimination (PD) for the situation?	Market segmentation (3rd degree PD); Two-part tariff or lump sum; Multi-product bundling; Individual-level 2nd degree PD; 1st degree PD → 7

Reference: McElwee, F., Cole, A., Kaliappan, G., Masters, A. and Steuten, L., 2023. HPR116 Is Payment Innovation Keeping Up With Therapy Innovation? A New Taxonomy of Innovative Payment Solutions to Aid Effective Implementation. Value in Health, 26(12), p.S274.
ISPOR Europe 2023 Poster Presentation. Journal publication forthcoming

Dealing with value uncertainty: aligning payment with outcome

… at the **population-level**

- Coverage with evidence development
  - Plausibly cost-effectiveness but significant uncertainty
  - Example: Cancer Drugs Fund (CDF) and Innovative Medicines Fund (IMF) in England

… at the **patient-level**

- Outcome-based payment
  - Payment only when a medicine works as intended (or rebate if it doesn't)
  - Example: Luxturna for inherited retinal disease in US[1]

EXPECTED BENEFITS

- ✓ Address decision uncertainty for payers
- ✓ Earlier access for patients
- ✓ Improve evidence base

CHALLENGES

- ❖ CED: Will it solve the uncertainty?
- ❖ Selecting the right outcomes
- ❖ Appropriate timeframe
- ❖ Data collection, infrastructure & governance

[1]Spark Therapeutics. Spark Therapeutics Announces First-of-their-kind Programs to Improve Patient Access to LUXTURNATM (voretigene neparvovec-rzyl), a One-time Gene Therapy Treatment – Spark Therapeutics. Accessed October 23, 2023.

Managing affordability or budget risk

… because of high up-front costs, a treatment could be unaffordable even if cost-effective

- Instalments
  - Spread cost over time
  - Example: Zolgensma for Spinal Muscular atrophy in US[1] and Italy[2]

… because of unknown prevalence or uptake

- Subscription agreements (“Netflix” model)
  - Fixed payment to the manufacturers, regardless of the number of patients treated
  - Example: Direct-acting antiviral (DAA) agents for Hepatitis C in Australia[3]

EXPECTED BENEFITS

- ✓ Mitigate short-term budget blow
- ✓ Predictable revenue stream for drug maker
- ✓ Prevent drug costs growing uncontrollably

CHALLENGES

- ❖ Agreeing on the terms of contract
- ❖ Aligning payment terms with value
- ❖ Insurance markets: adverse selection & exclusions

[1]Novartis. AveXis Announces Innovative Zolgensma® Gene Therapy Access Programs for US Payers and Families. Novartis. Published May 24, 2019. Accessed July 27, 2023.
[2]ATMP forum. Quarto Report Italiano Sulle Advanced Therapy Medicinal Product.; 2021. https://www.atmpforum.com/wp-content/uploads/2021/11/IVReportATMPForum_Nov2021.pdf
[3]Moon S, Erickson E. Universal Medicine Access through Lump-Sum Remuneration — Australia’s Approach to Hepatitis C. N Engl J Med. 2019;380(7):607-610. doi:10.1056/NEJMp1813728

We'll probably need to mix & match

Alzheimers Dement. 2020 Nov;16(11):1568-1570. doi: 10.1002/alz.12155. Epub 2020 Aug 18.

Preparing the health-care system to pay for new Alzheimer's drugs

Pei-Jung Lin [1], Joshua T Cohen [1], Peter J Neumann [1]

Affiliations + expand

PMID: 32808733 PMCID: PMC7666042 DOI: 10.1002/alz.12155

Performance warranty + subscription payment agreement

Value-based subscription model

Value-based milestone contract with a volume cap

Multi-year stop loss policies with pass-through warranties

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Managing the Challenges of Paying for Gene Therapy: Strategies for Market Action and Policy Reform

April 23, 2024

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Key Facilitators

- **Developing best practices** for outcome measurement
- **Real-world data**
  - Infrastructure to track outcomes
  - Information governance: clear, ethical and enabling standards for data confidentiality, access and use.
  - Data linkage and integration of health data and payment platforms
- **Policy reforms** may be needed

Most important: Agreement across stakeholders that it's the best thing to do

Conclusion

For patients, healthcare systems, and societies to benefit from the potential of high-value treatments, we need to evolve the way we pay for them.

- Early treatments for chronic progressive disease present challenges for value assessment, uncertainty and affordability.
- We can't rely on traditional approaches: alternative payment models must be leveraged to enable earlier access while addressing uncertainty and mitigating budget risk.
- Selection of the right payment model must be fit for purpose:
  are we solving for financial or evidential challenges?
- Real-world data are critical enablers *for assessing outcomes and fostering a learning healthcare system.*

Discussion

Discussion / Q & A

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Improving healthcare decisions

Thank you!

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