Health Equity

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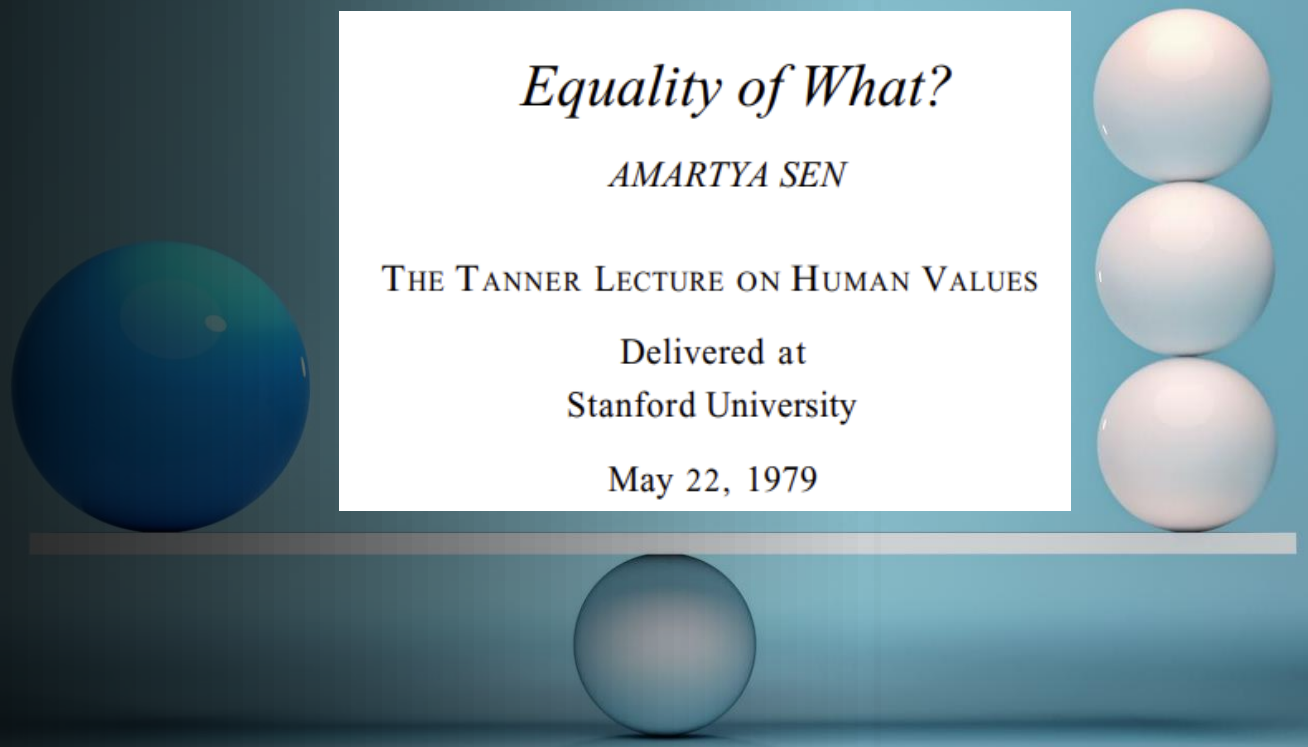
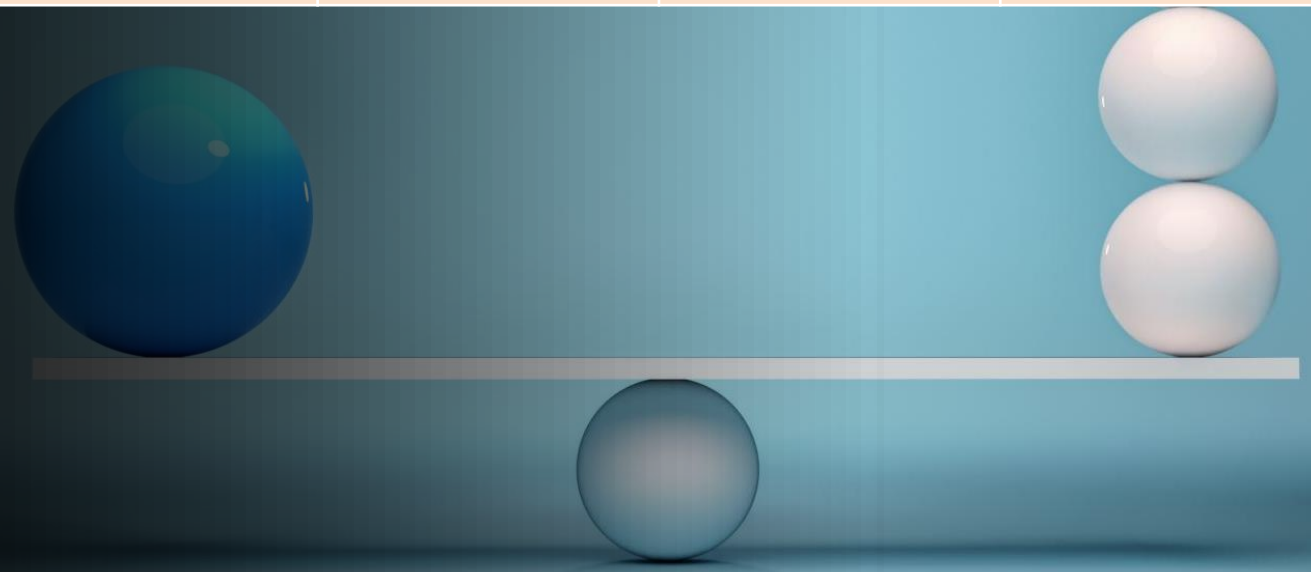
Equity ≠ Equality

Equity = Some Equality



Populations	Base Total Health	INB of Treatment	Total Health if Treatment Adopted
A	$H_A(0)$	$ICER_A(0)$	$H_A(1)$
B	$H_B(0)$	$ICER_B(0)$	$H_B(1)$
C	$H_C(0)$	$ICER_C(0)$	$H_C(1)$



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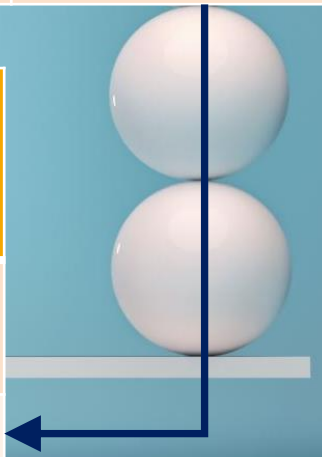
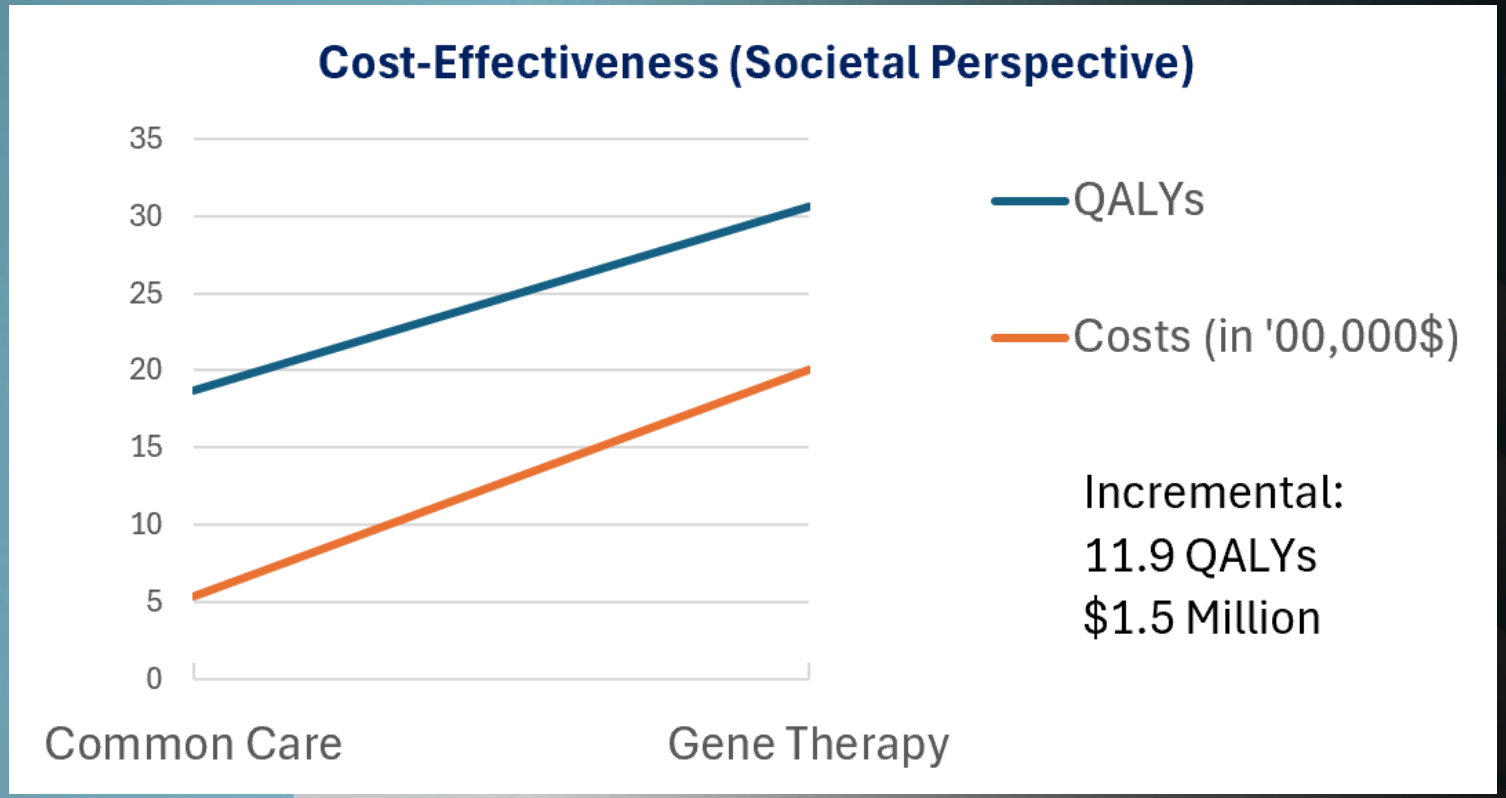
Social Welfare Criterion	Approach	Implications for optimal allocation across population
Maximize: Sum{H(1)s – H(0)s}	Utilitarian	Equal/Close marginal values or ICERs
Maximize: H(1)s – H(0)s | Min(H(0))	Rawlsian	Only care about worst-off
Minimize: Variance (H(1)s)	Difference (Leximin) Principle	Equal/Close H(1)s
Maximize: Sum{ ω(H(1)s – H(0)s | H(0)s)} , where ω() = weights	Atkinson, Generalized Entropy, Gini	Equal/Close socially weighted marginal values or ICERs



Gene Therapy

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

Background: Sickle cell disease (SCD) and its complications contribute to high rates of morbidity and early mortality and high cost in the United States and African heritage community.

Objective: To evaluate the cost-effectiveness of gene therapy for SCD and its value-based prices (VBPs).

Design: Comparative modeling analysis across 2 independently developed simulation models (University of Washington Model for Economic Analysis of Sickle Cell Cure [UW-MEASURE] and Fred Hutchinson Institute Sickle Cell Disease Outcomes Research and Economics Model [FH-HISCORE]) using the same databases.

Data Sources: Centers for Medicare & Medicaid Services claims data, 2008 to 2016; published literature.

Target Population: Persons eligible for gene therapy.

Time Horizon: Lifetime.

Perspective: U.S. health care sector and societal.

Intervention: Gene therapy versus common care.

Outcome Measures: Incremental cost-effectiveness ratios (ICERs), equity-informed VBPs, and price acceptability curves.

Results of Base-Case Analysis: At an assumed $2 million price for gene therapy, UW-MEASURE and FH-HISCORE estimated ICERs of $193 000 per QALY and $427 000 per QALY, respectively, under the health care sector perspective. Corresponding estimates from the societal perspective were $126 000 per QALY and $281 000 per QALY. The difference in results between models stemmed primarily from considering a slightly different target population and incorporating the quality-of-life (QOL) effects of splenic sequestration, priapism, and acute chest syndrome in the UW model. From a societal perspective, acceptable (>90% confidence) VBPs ranged from $1 million to $2.5 million depending on the use of alternative effective metrics or equity-informed threshold values.

Results of Sensitivity Analysis: Results were sensitive to the costs of myeloablative conditioning before gene therapy, effect on caregiver QOL, and effect of gene therapy on long-term survival.

Limitation: The short-term effects of gene therapy on vaso-occlusive events were extrapolated from 1 study.

Conclusion: Gene therapy for SCD below a $2 million price tag is likely to be cost-effective when applying a societal perspective at an equity-informed threshold for cost-effectiveness analysis.

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Apply Atkinson Social Welfare Function

EDE = Equally Distributed Equivalent = the population-wide equity weighted health

$$= \left[\left(\frac{N1}{(N1+N2)} \right) \cdot (QALYS_{SCD})^{(1-\epsilon)} + \left(\frac{N2}{(N1+N2)} \right) \cdot (QALYS_{GEN})^{(1-\epsilon)} \right]^{\left(1/(1-\epsilon)\right)}$$

ϵ= the inequality aversion parameter; N1= SCD target population; N2= General Population

General Population QALYs with SCD gene therapy =

General Population QALY with no SCD Gene therapy – (N1* $1,498,971/(λ*N2)

λ = CEA threshold

	Population Size	Population Proportions
N1 (Target SCD)	5000	0.000015
N2 (General)	330000000	0.999985

Keep Traditional Threshold, Inequality Aversion

Threshold	**100000**
Inequality aversion (ϵ)	**0.9**
Without gene therapy	
SCD pop QALYS	42.7
General pop QALYS	65
EDE	64.99959476
With gene therapy	
SCD pop QALYS	54.6
General pop QALYS	64.9998
EDE	64.99960267
Diff in EDE	**7.90746E-06**

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Annals of Internal Medicine ORIGINAL RESEARCH

Distributional Cost-Effectiveness of Equity-Enhancing Gene Therapy in Sickle Cell Disease in the United States

George Goshua, MD, MSc; Cecelia Calhoun, MD, MBA, MPH; Satoko Ito, MD, PhD; Lyndon P. James, MBBS, MPH; Andrea Luviano, MD, MPH; Lakshmanan Krishnamurti, MD; and Ankur Pandya, PhD

Hurley et al. JHE 2020: $\epsilon = 1.17$
Glassman US Census 2017: $\epsilon = 0.5, 1.0, 2.0$

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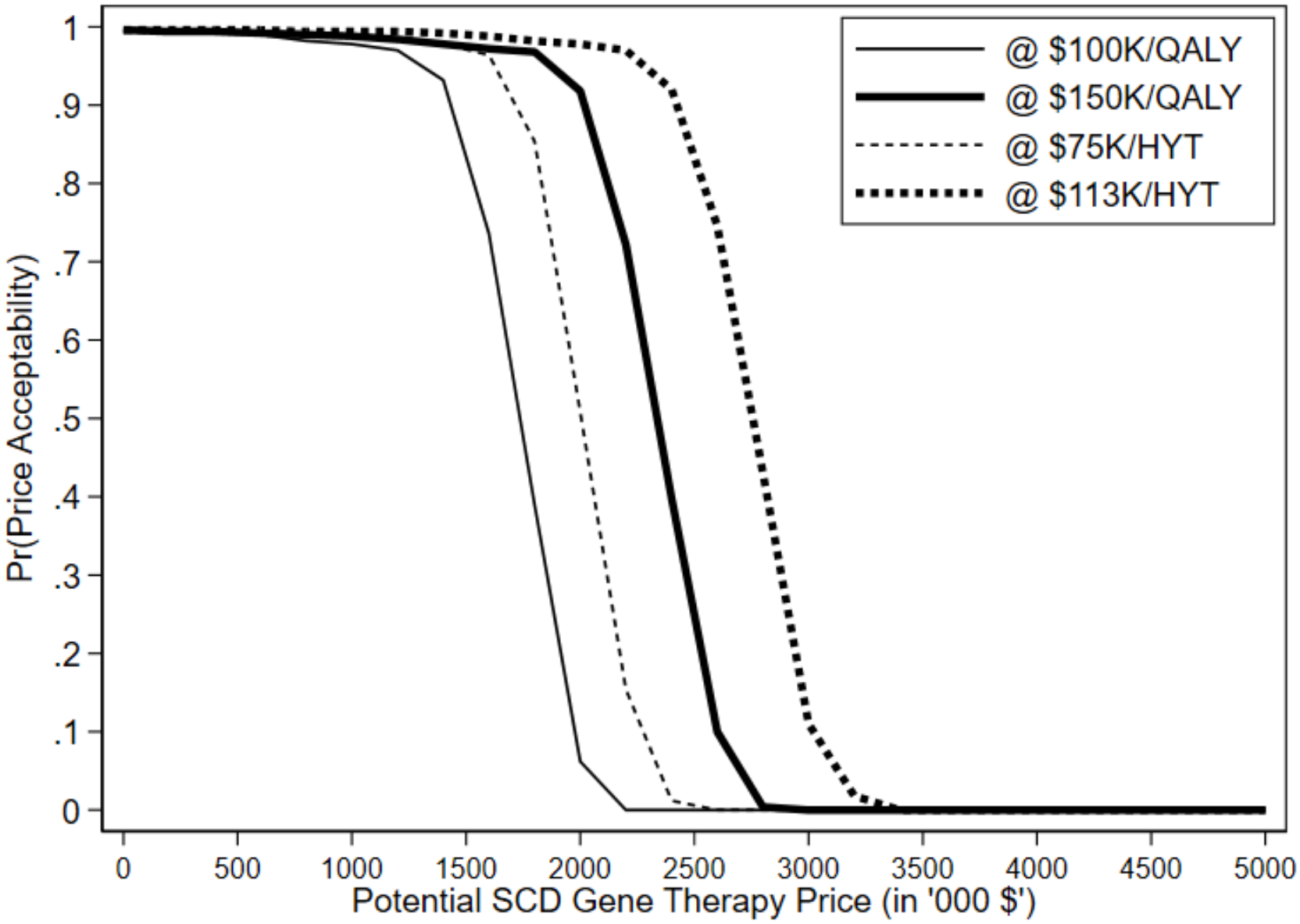
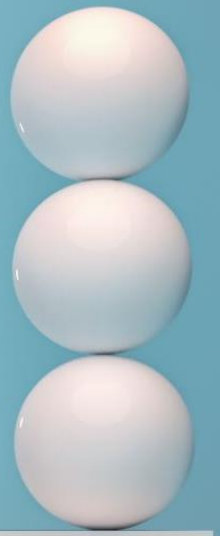
Change Threshold, Keep Inequality Aversion to Zero

Threshold	**142175**
Inequality aversion (ϵ)	**0**
Without gene therapy	
SCD pop QALYS	42.7
General pop QALYS	65
EDE	64.99966213
With gene therapy	
SCD pop QALYS	54.6
General pop QALYS	64.9998
EDE	64.99966984
Diff in EDE	**7.90809E-06**





PRICE ACCEPTABILITY CURVES

Health Years in Total (HYT)

QALYS

- Multiplicative in QOL and LE
  - QOL*LE
- Basis in expected utility theory
- Violates IRA requirements – values life extension of poor QOL individuals lower than better QOL individuals
- Have proportional tradeoff property for QOL elicitation (TTO)
- Does not directly address severity-based distributional issues

HYT

- Additive in QOL and LE -
  - QOL evaluated with Max LE under any treatment
  - LE evaluated at perfect QOL (=1)
- Basis in reference-dependent utility
- Does not violate IRA requirement
- Maintains property to elicit QOL through TTOs
- Does not directly address severity-based distributional issues

Detail discussions of HYT can be found in Tuesday session, "I have a better QALY than you"

Conclusions

- Incorporating Health Equity in CEA can be achieved in different ways
- It is important to do this in a transparent way
- Debates exist about whether to codify these impacts through specific parameters or a deliberative process.
- The answer lies with decision-makers, not analysts.