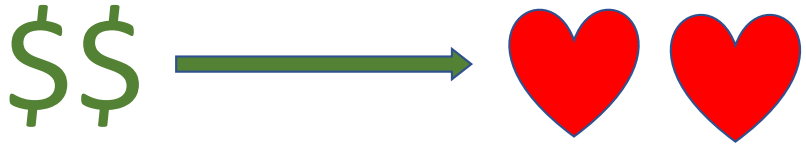
Equity-informative cost-effectiveness analysis

Ankur Pandya, PhD
Associate Professor of Health Decision Science
Department of Health Policy and Management
Harvard T.H. Chan School of Public Health

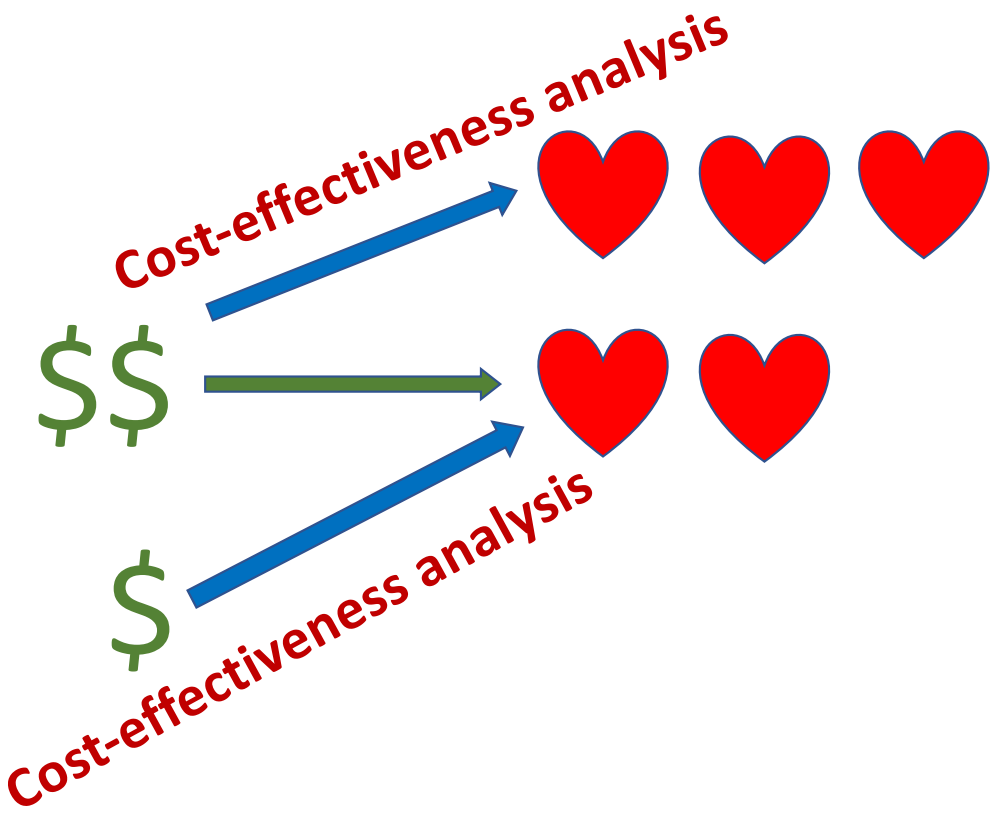
Equity-informative cost-effectiveness analysis

Ankur Pandya, PhD
Associate Professor of Health Decision Science
Department of Health Policy and Management
Harvard T.H. Chan School of Public Health

Starting premise for CEA: we put money into the health system to generate health outcomes



Cost-effectiveness analysis can be used to get more “bang for buck” in population health



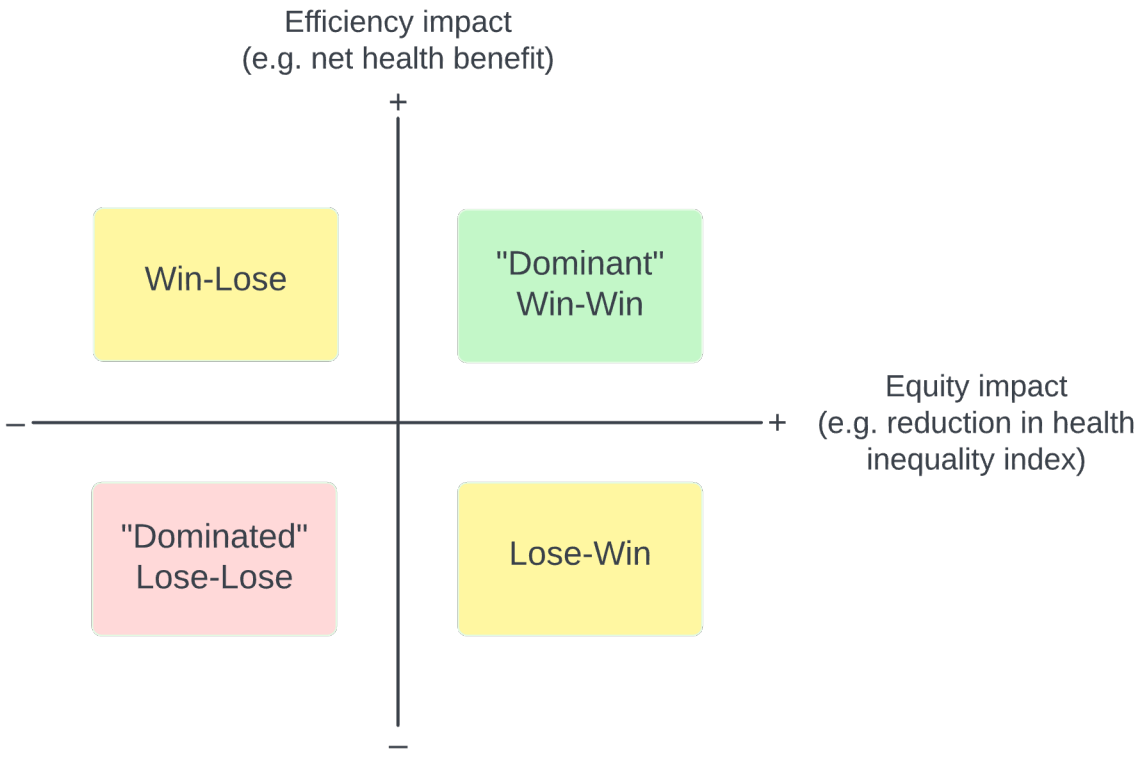
No health decision scientist believes CEA is the only input to a good policy decision-making process

- **Cost-effectiveness analysis** tells us how to maximize population health given a budget constraint (i.e., **tells us about efficiency**)
  - "**A QALY is a QALY is a QALY**"
- But decision-makers will also care about:
  - The **distribution** of these health and cost outcomes
  - **Other outcomes** (e.g., financial risk protection)
  - **Fair process** (closing argument: CEA an input into a fair process)

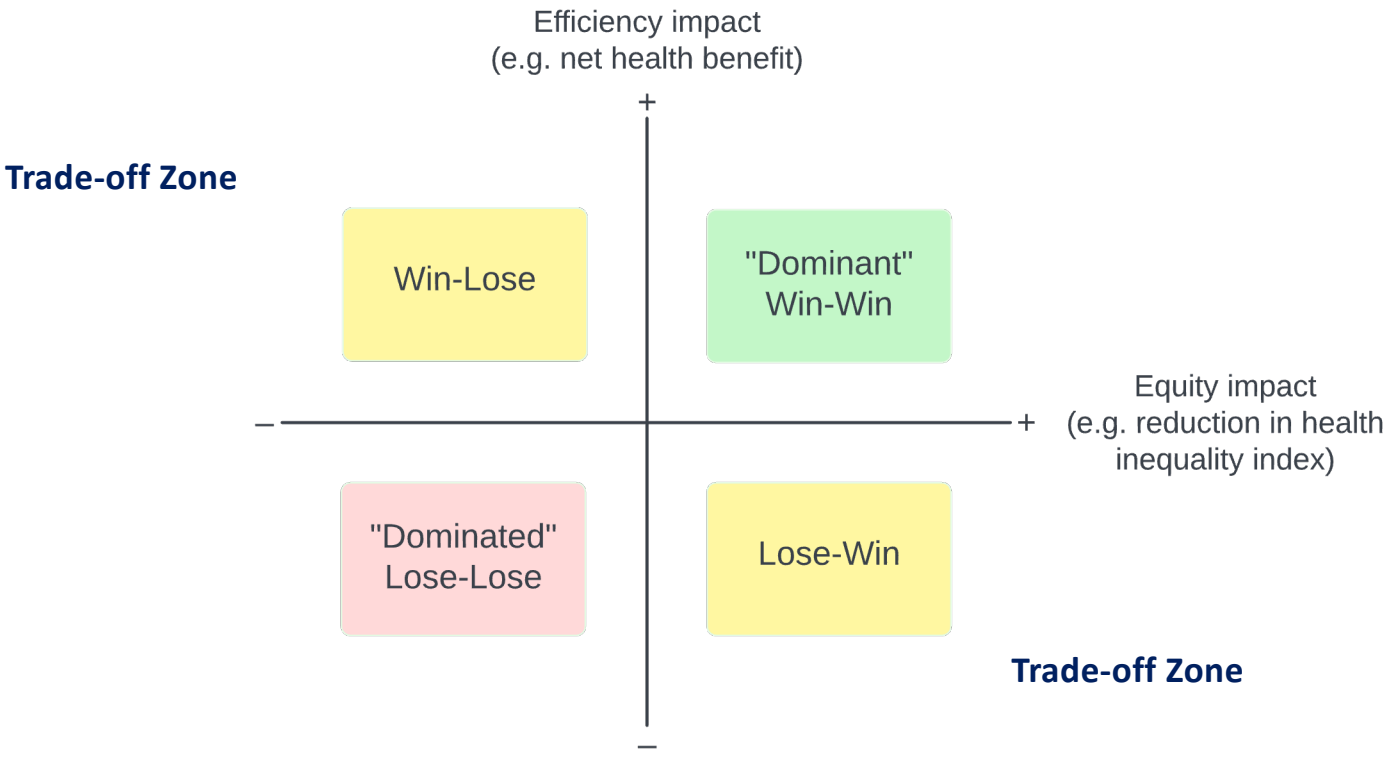
No health decision scientist believes CEA is the only input to a good policy decision-making process

- **Cost-effectiveness analysis** tells us how to maximize population health given a budget constraint (i.e., **tells us about efficiency**)
  - "**A QALY is a QALY is a QALY**"
- But decision-makers will also care about:
  - The **distribution** of these health and cost outcomes
  - **Other outcomes** (e.g., financial risk protection)
  - **Fair process** (closing argument: CEA an input into a fair process)

Equity-efficiency plane



Equity-efficiency plane



Equity-informative CEA methods have been proposed to analyze the trade-offs between equity and efficiency…

PharmacoEconomics (2016) 34:913–923
DOI 10.1007/s40273-016-0414-z

PRACTICAL APPLICATION

Extended Cost-Effectiveness Analysis for Health Policy Assessment: A Tutorial

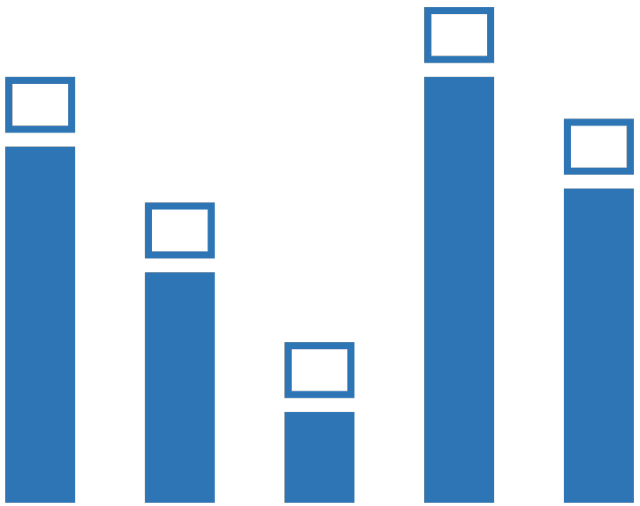
Stéphane Verguet[1] · Jane J. Kim[2] · Dean T. Jamison[3,4]

TUTORIAL

Distributional Cost-Effectiveness Analysis: A Tutorial

Miqdad Asaria, MSc, Susan Griffin, PhD, Richard Cookson, PhD

Distributional cost-effectiveness analysis (DCEA) is a framework for incorporating health inequality concerns into the economic evaluation of health sector interventions. In this tutorial, we describe the technical details of how to conduct DCEA, using an illustrative example comparing alternative ways of implementing the National Health Service (NHS) Bowel Cancer Screening Programme (BCSP). The 2 key stages in DCEA are 1) modeling social distributions of health associated with different interventions, and 2) evaluating social distributions of health with respect to the dual objectives of improving total population health and reducing unfair health inequality. As well as describing the technical methods used, we also identify the data requirements and the social value judgments that have to be made. Finally, we demonstrate the use of sensitivity analyses to explore the impacts of alternative modeling assumptions and social value judgments. **Key words:** *cost-effectiveness analysis; economic evaluation; efficiency; equality; equity; fairness; health distribution; health inequality; inequality measures; opportunity cost; social value judgments; social welfare functions; tradeoff.*
(Med Decis Making 2016;36:8–19)



Distributional Cost-Effectiveness Analysis

DCEA

- Cost-effectiveness analysis (CEA) does not add any additional weight based on who experiences health benefits or costs
  - What if it is more cost-effective to provide additional health benefits to healthy people as opposed to sick individuals?
  - Or rich vs. poor populations?
  - Urban vs. rural?

DCEA

- Cost-effectiveness analysis (CEA) does not add any additional weight based on who experiences health benefits or costs
  - What if it is more cost-effective to provide additional health benefits to healthy people as opposed to sick individuals?
  - Or rich vs. poor populations?
  - Urban vs. rural?
- Distributional cost-effectiveness analysis (DCEA)
  - is a framework that incorporates health inequality impacts into CEA
  - assigns “equity weights”, although these can be difficult to estimate for reasons related to both theory and practice

Distributional Cost-Effectiveness Analysis: A Tutorial

Miqdad Asaria, MSc, Susan Griffin, PhD, Richard Cookson, PhD

Distributional cost-effectiveness analysis (DCEA) is a framework for incorporating health inequality concerns into the economic evaluation of health sector interventions. In this tutorial, we describe the technical details of how to conduct DCEA, using an illustrative example comparing alternative ways of implementing the National Health Service (NHS) Bowel Cancer Screening Programme (BCSP). The 2 key stages in DCEA are 1) modeling social distributions of health associated with different interventions, and 2) evaluating social distributions of health with respect to the dual objectives of improving total population health and reducing unfair health inequality. As well as describing the technical methods used, we also identify the data requirements and the social value judgments that have to be made. Finally, we demonstrate the use of sensitivity analyses to explore the impacts of alternative modeling assumptions and social value judgments. ***Key words:*** *cost-effectiveness analysis; economic evaluation; efficiency; equality; equity; fairness; health distribution; health inequality; inequality measures; opportunity cost; social value judgments; social welfare functions; tradeoff.* ***(Med Decis Making 2016;36:8–19)***

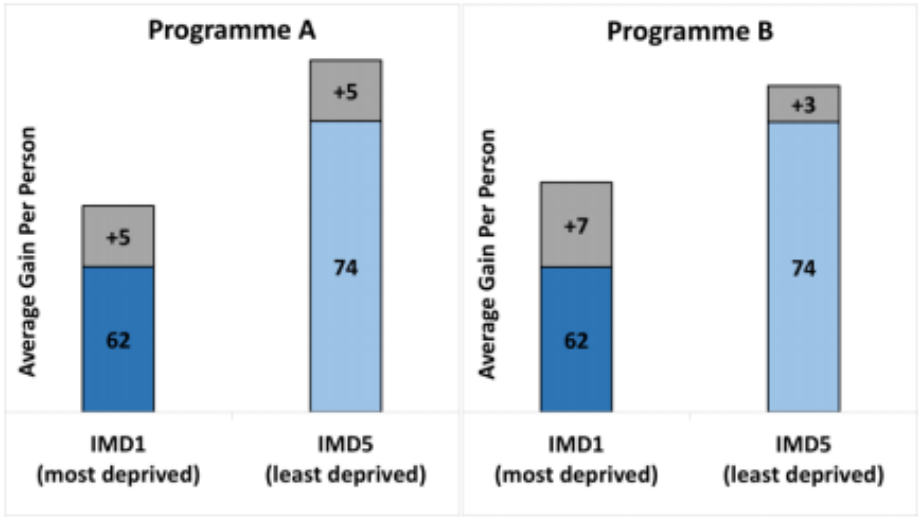
The key input that differentiates DCEA from conventional CEA is some quantitative estimate of society's inequality aversion

a) Inequality aversion

Researchers have conducted an online survey of the general public in England to ask about how much they care about reducing inequality between rich and poor groups compared to improving overall health (see **Appendix 2**) [2]. The survey includes questions asking respondents to choose between two programmes that cost the same but with different health benefits for people living in the most deprived (IMD1) and those living the least deprived (IMD5) areas. A typical question in this survey is as below.

Which programme should the government choose?

- **Programme A** ☐
- **Programme A and B are equally good** ☐
- **Programme B** ☐



EDE health

b) Equally distributed equivalent (EDE) health

We assign this parameter value of ε to the health in each group to calculate the weighted health for the whole population using the mathematic algorithm below. The weighted health is called **'equally distributed equivalent' (EDE)** health. EDE takes into account the health level and the population size of each socioeconomic group and allows us to compare the effects between the interventions.

$$EDE = \left(\frac{1}{N}\sum h_i^{1-\varepsilon}\right)^{\frac{1}{1-\varepsilon}}$$

N=population size, hi=health in each group
ε=inequality aversion index

When no smoking cessation services are provided on the NHS, the EDE health is 69.47 years. This means considering the inequality aversion, the health is equivalent to that each IMD group has the life expectancy of 69.47 years in full health.

EDE health

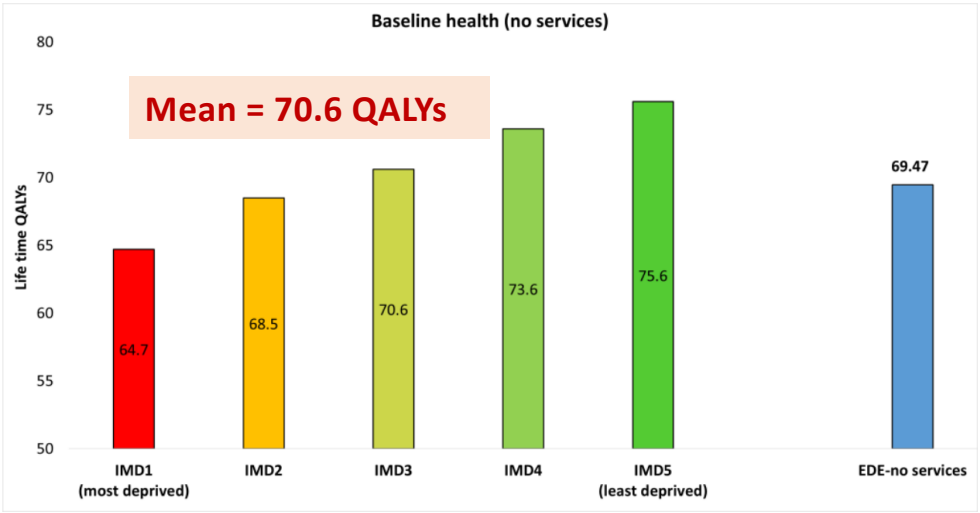
b) Equally distributed equivalent (EDE) health

We assign this parameter value of ε to the health in each group to calculate the weighted health for the whole population using the mathematic algorithm below. The weighted health is called **'equally distributed equivalent' (EDE)** health. EDE takes into account the health level and the population size of each socioeconomic group and allows us to compare the effects between the interventions.

$$EDE = \left(\frac{1}{N}\sum h_i^{1-\varepsilon}\right)^{\frac{1}{1-\varepsilon}}$$

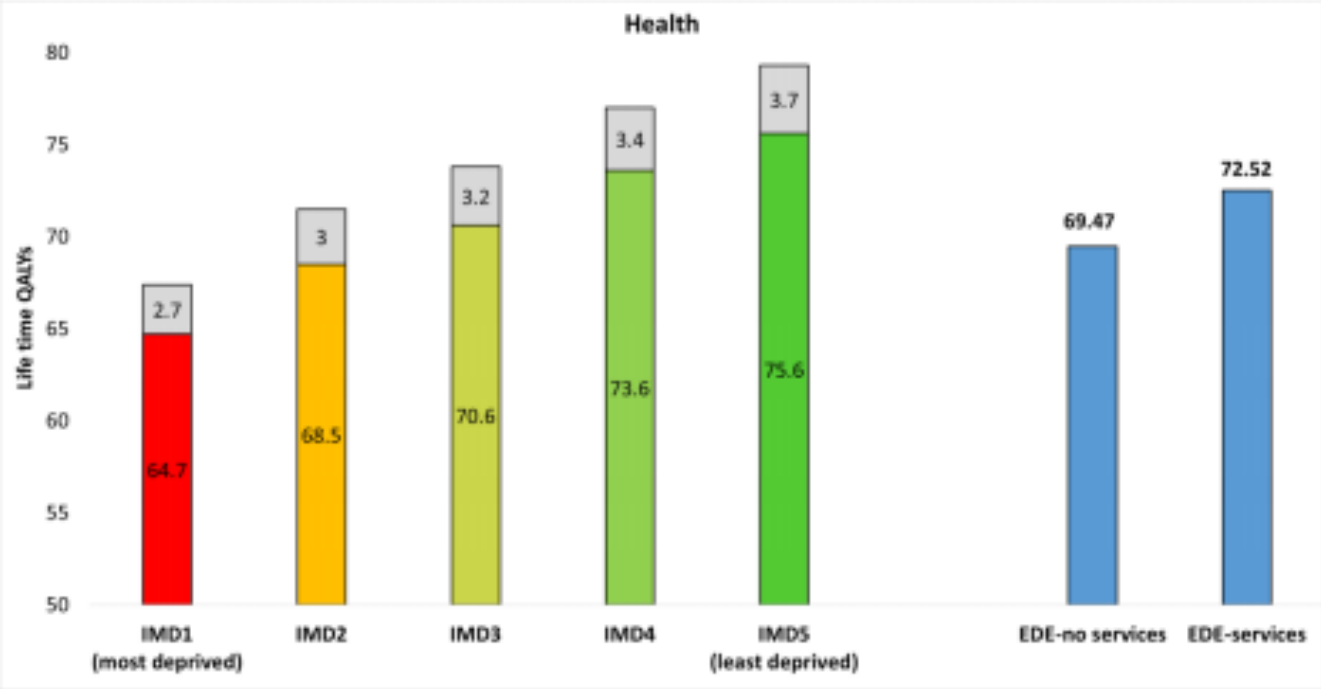
N=population size, hi=health in each group
ε=inequality aversion index

When no smoking cessation services are provided on the NHS, the EDE health is 69.47 years. This means considering the inequality aversion, the health is equivalent to that each IMD group has the life expectancy of 69.47 years in full health.



The decision rule in a DCEA is to choose the intervention with the highest EDE

We can also calculate the EDE health if smoking cessation services were provided. The EDE health with services is 72.52 years in full health.



Pioneering Gene Therapy Freed Her of Sickle Cell. Is a Cure at Hand?

Such treatments are extraordinarily promising and costly. Will the Biden administration commit to spending that could speed clinical trial results?

By Gina Kolata

Published Sept. 14, 2021 Updated Nov. 4, 2021

Helen Obando, at home in Mesa, Ariz. An experimental gene therapy she received last year successfully rid her of her sickle cell disease, eliminating her intense suffering and transforming her into a teenager like any other. Ash Ponders for The New York Times

Gene therapy cure (priced at $2.2 million) not cost-effective using conventional CEA methods

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

Background: Gene therapy is a potential cure for sickle cell disease (SCD). Conventional cost-effectiveness analysis (CEA) does not capture the effects of treatments on disparities in SCD, but distributional CEA (DCEA) uses equity weights to incorporate these considerations.

Objective: To compare gene therapy versus standard of care (SOC) in patients with SCD by using conventional CEA and DCEA.

Design: Markov model.

Data Sources: Claims data and other published sources.

Target Population: Birth cohort of patients with SCD.

Time Horizon: Lifetime.

Perspective: U.S. health system.

Intervention: Gene therapy at age 12 years versus SOC.

Outcome Measures: Incremental cost-effectiveness ratio (ICER) (in dollars per quality-adjusted life-years [QALYs] gained) and threshold inequality aversion parameter (equity weight).

Results of Base-Case Analysis: Gene therapy versus SOC for females yielded 25.5 versus 15.7 (males: 24.4 vs. 15.5) discounted lifetime QALYs at costs of $2.8 million and $1.0 million (males: $2.8 million and $1.2 million), respectively, with an ICER of $176 000 per QALY (full SCD population). The inequality aversion parameter would need to be 0.90 for the full SCD population for gene therapy to be preferred per DCEA standards.

Results of Sensitivity Analysis: SOC was favored in 100.0% (females) and 87.1% (males) of 10 000 probabilistic iterations at a willingness-to-pay threshold of $100 000 per QALY. Gene therapy would need to cost less than $1.79 million to meet conventional CEA standards.

Limitation: Benchmark equity weights (as opposed to SCD-specific weights) were used to interpret DCEA results.

Conclusion: Gene therapy is cost-ineffective per conventional CEA standards but can be an equitable therapeutic strategy for persons living with SCD in the United States per DCEA standards.

Primary Funding Source: Yale Bernard G. Forget Scholars Program and Bunker Endowment.

Ann Intern Med. doi:10.7326/M22-3272 **Annals.org**
For author, article, and disclosure information, see end of text.
This article was published at Annals.org on 30 May 2023.

But in our DCEA approach, the threshold ε~0.9

(i.e., that's equity weight where EDE of gene therapy = EDE of standard of care)

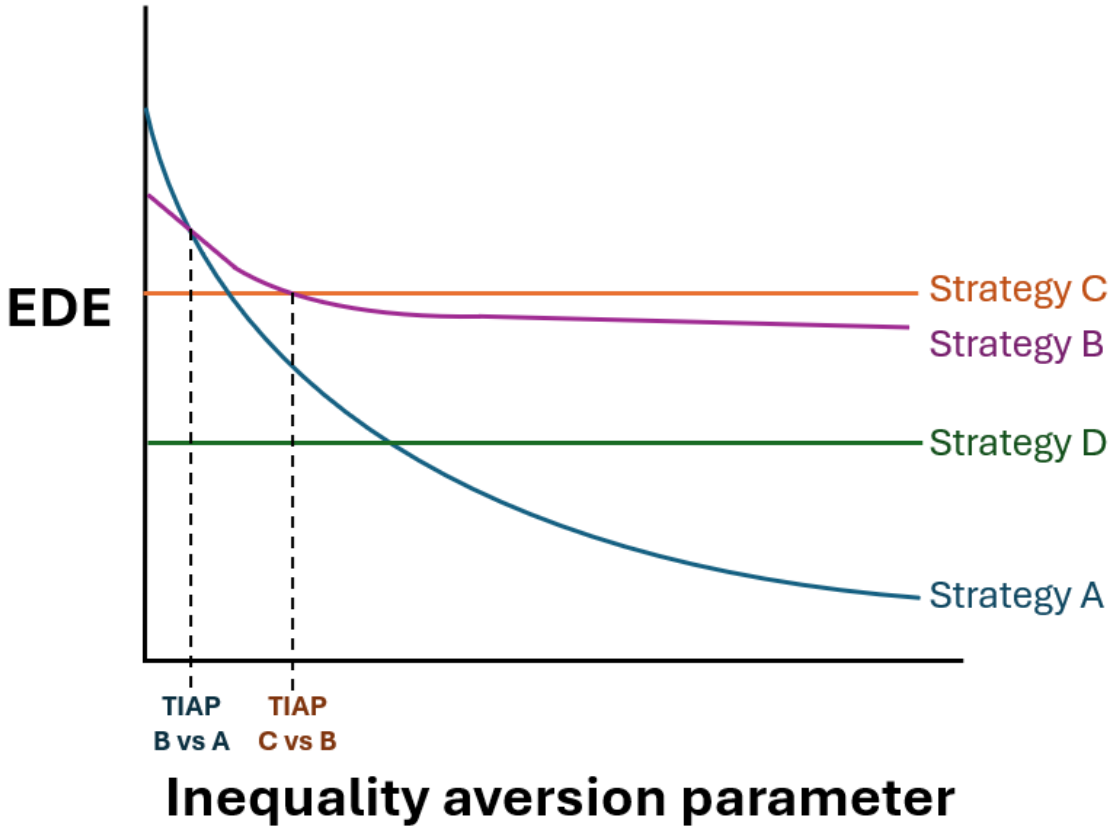
Table 2. Base-Case Results and Probabilistic Sensitivity Analysis

Variable	Standard of Care	Gene Therapy
Cost, *US $**	1 120 000	2 770 000
QALYs*	15.6	25.0
ICER, *US $ per QALY**	–	176 000
95% credible interval for ICER, *US $ per QALY*		
Females	–	155 000–208 000
Males	–	14 800–243 000
Threshold inequality aversion parameter (equity weight)*	–	0.90

ICER = incremental cost-effectiveness ratio; QALY = quality-adjusted life-year.

* Lifetime, discounted, per-person results for full population.

What to do if we don't know ε? Introducing the **threshold inequality aversion parameter (TIAP)!**



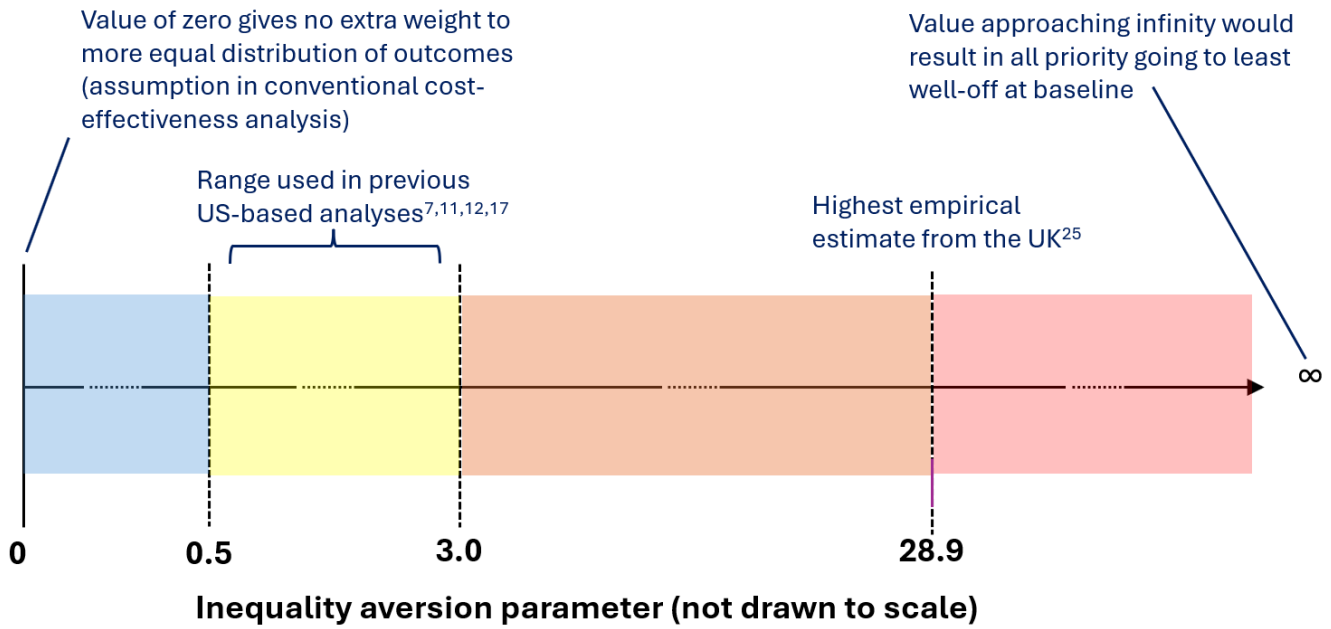
How do we interpret the TIAP? First we need a sense of what ε values have been estimated or used previously (1/2)…

Study (Author, Year)	Country	Method or Convention	Context or Study Design	Equity-Relevant Sub-Groups	Estimated/Applied Inequality Aversion Parameter
Levy, 2006[12]	US	Convention, "typical values applied in the literature"	Environmental regulation	Not specified, mentions income and health	0.25-2.0
Kawachi, 1997[11]	US	Convention, "following previous work (Atkinson, 1970; Sen, 1973; Cowell, 1977)"	Correlation of income distribution to mortality	Income distribution	0.5-2.0
Regidor, 2003[15]	Spain	Convention, cites Atkinson, 1970; Sen, 1973	Association of income inequality and life expectancy	Income distribution	1.0-2.0
Aristei, 2011[16]	Italy	Convention, "a range which is commonly adopted in empirical analyses."	Distribution of wellbeing across Italy	Regional wellbeing measures	0.3-3.0
Glassman, 2019[7]	US	Convention, to show a range of "relatively low and relatively high levels of inequality aversion"	Applying various inequality measures to different dimensions using previously collected survey data	Multi-dimensional inequality including income, housing, health, education, leisure, vehicle ownership	0.5-3.0
Cropper, 2016[17]	US	Hypothetical choice survey	Non-representative online survey (n=913)	Distribution of health risks based on environmental exposures	0.72 (mean), 2.8 (median)
Hurley, 2020[18]	Canada	Hypothetical choice survey	Randomly selected community-based residents of Ontario (n=1,964)	Health across income quintiles	Median value slightly greater than 3.0

How do we interpret the TIAP? First we need a sense of what ε values have been estimated or used previously (2/2)…

Study (Author, Year)	Country	Method or Convention	Study Design or Context	Equity-Relevant Sub-Groups	Estimated/Applied Inequality Aversion Parameter
Pinho, 2018[19]	Portugal	Hypothetical choice survey	Self-administered survey completed by college students (n=422)	Health by socioeconomic class	Plausible range of 2.24 to 4.85
Robson, 2024[20]	UK	Hypothetical choice survey	Representative sample online survey (n=337)	Health inequality by income	6.5 (approximation of their median value)
Edlin, 2012[21]	UK	Hypothetical choice survey	Face-to-face interviews with members of the general public in their homes (n=559)	Health inequality	6.8 or greater
Ali, 2017[22]	UK	Hypothetical choice survey	Face-to-face interviews (n=52) and online survey (n=83) of general public	Health by income groups	10.87
Robson, 2016[23]	UK	Hypothetical choice survey	Online survey of general public (n=244)	Health by income groups	11.0 (median)
Boujaoude, 2025[24]	Australia	Hypothetical choice survey	Online survey of representative sample (n=2,383)	Health by income, ethnic, and geographic sub-groups	27.16 (median by income), 17.73 (median by ethnic), 31.7 (median by geographic)
Dolan, 2010[25]	UK	Hypothetical choice survey	Face-to-face interviews of sample "broadly" of representative Yorkshire and Humberside region (n=130)	Health by social class	28.9

Then we can interpret the TIAP in the context of these values!



How to interpret threshold inequality aversion parameter (TIAP) for the US

- TIAP <0.5 strongly implies equity-improving strategy optimal
- TIAP 0.5-3.0 implies equity-improving strategy optimal, but more precise value could be needed
- TIAP 3.0-28.9 implies optimal strategy could depend on decision context, or more precise value needed
- TIAP >28.9 strongly implies equity-improving strategy not optimal

Pros and cons of DCEA

- Incorporates inequality concerns into CEA
- General and flexible

- A number of value judgements need to be made when implementing DCEA
- DCEA is demanding in terms of data
- Results are complex; needs better ways to communicate findings to nonspecialist audiences
- Does not examine effects on financial risk protection.

Extended Cost-Effectiveness Analysis

Extended cost-effectiveness analysis

PharmacoEconomics (2016) 34:913–923
DOI 10.1007/s40273-016-0414-z

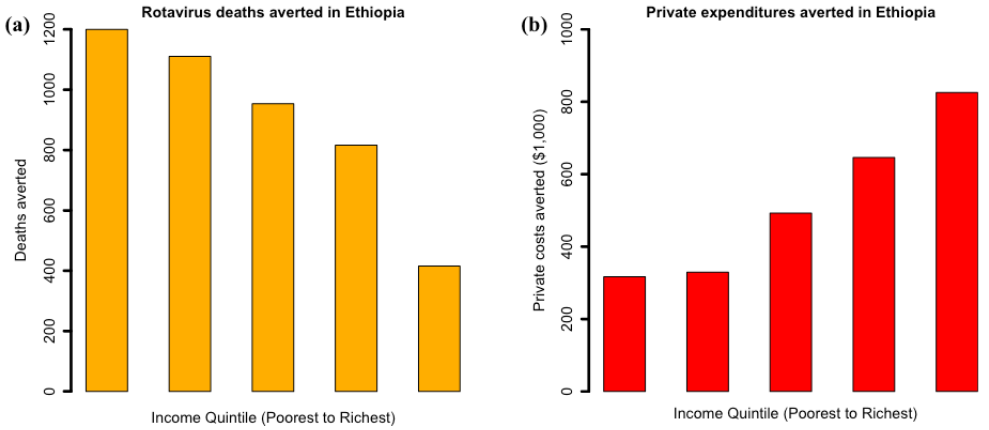
PRACTICAL APPLICATION

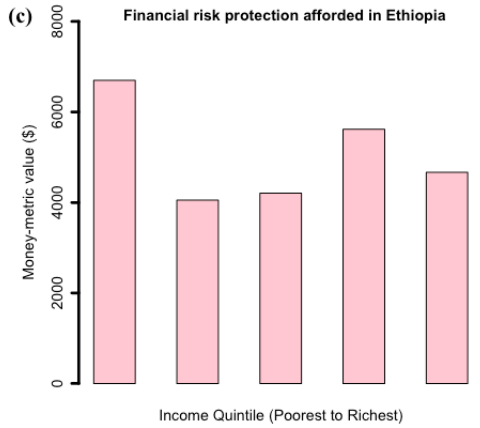
Extended Cost-Effectiveness Analysis for Health Policy Assessment: A Tutorial

Stéphane Verguet[1] · Jane J. Kim[2] · Dean T. Jamison[3,4]

Extended cost-effectiveness analysis

- Similar idea: disaggregating outcomes by equity-relevant subgroups
- Displays three outcomes by population stratum (subgroups)
  - Health gains
  - Private expenditures averted
  - Financial risk protection afforded





Applied ECEA Example

Evaluating efficiency and equity of prevention and control strategies for rheumatic fever and rheumatic heart disease in India: an extended cost-effectiveness analysis

Jyoti Dixit, Shankar Prinja, Gaurav Jyani, Pankaj Bahuguna, Ankur Gupta, Rajesh Vijayvergiya, Rajesh Kumar

Dixit J, Prinja S, Jyani G, Bahuguna P, Gupta A, Vijayvergiya R, Kumar R. Evaluating efficiency and equity of prevention and control strategies for rheumatic fever and rheumatic heart disease in India: an extended cost-effectiveness analysis. Lancet Glob Health. 2023 Mar;11(3):e445–55.

Conceptual framework

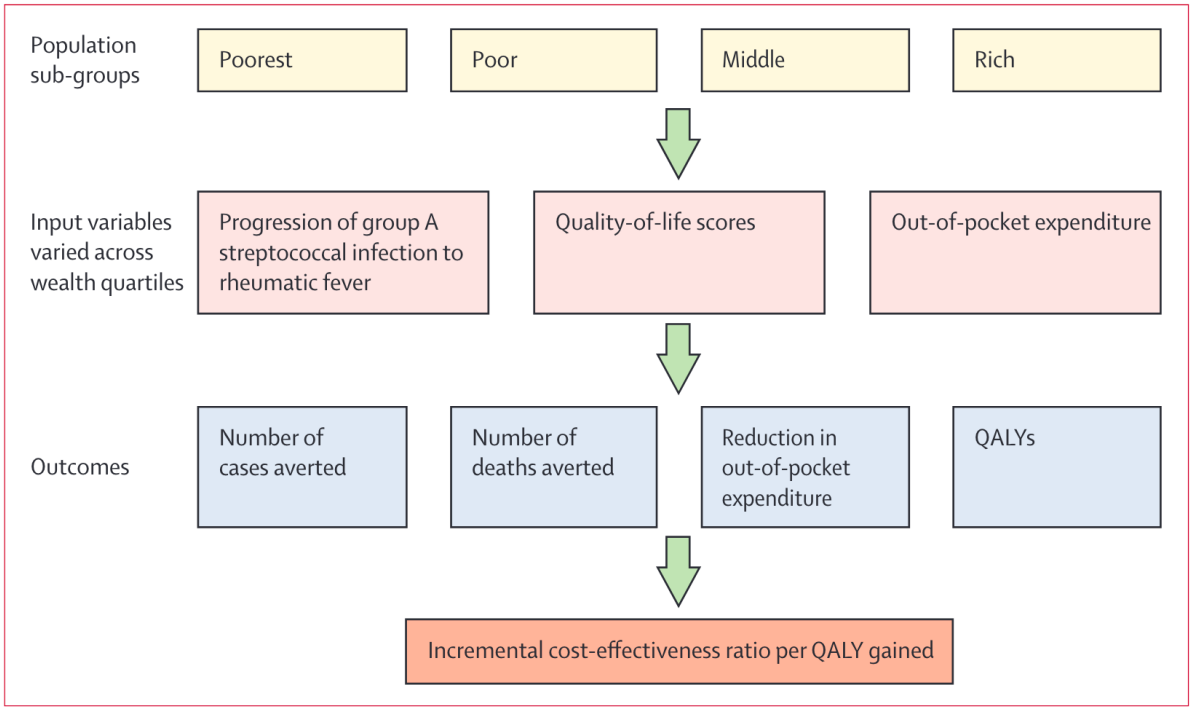


Figure 1: **Conceptual framework of the extended cost-effectiveness analysis**
QALY=quality-adjusted life-years.

Disaggregated health outcomes

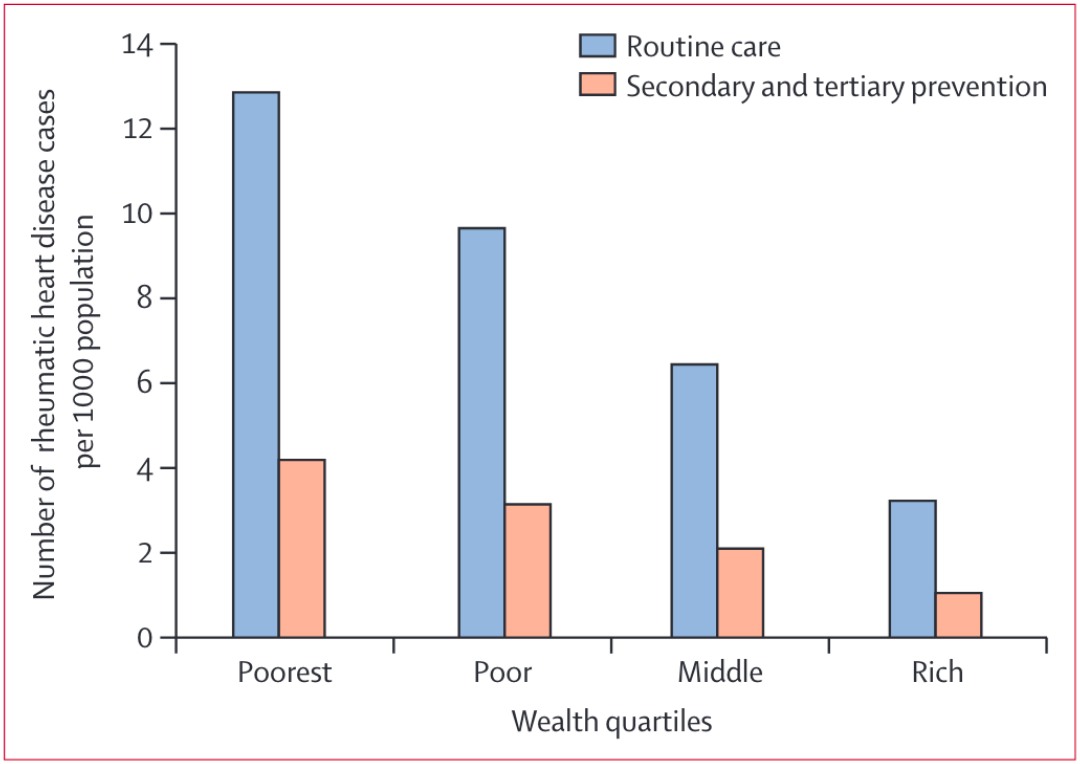
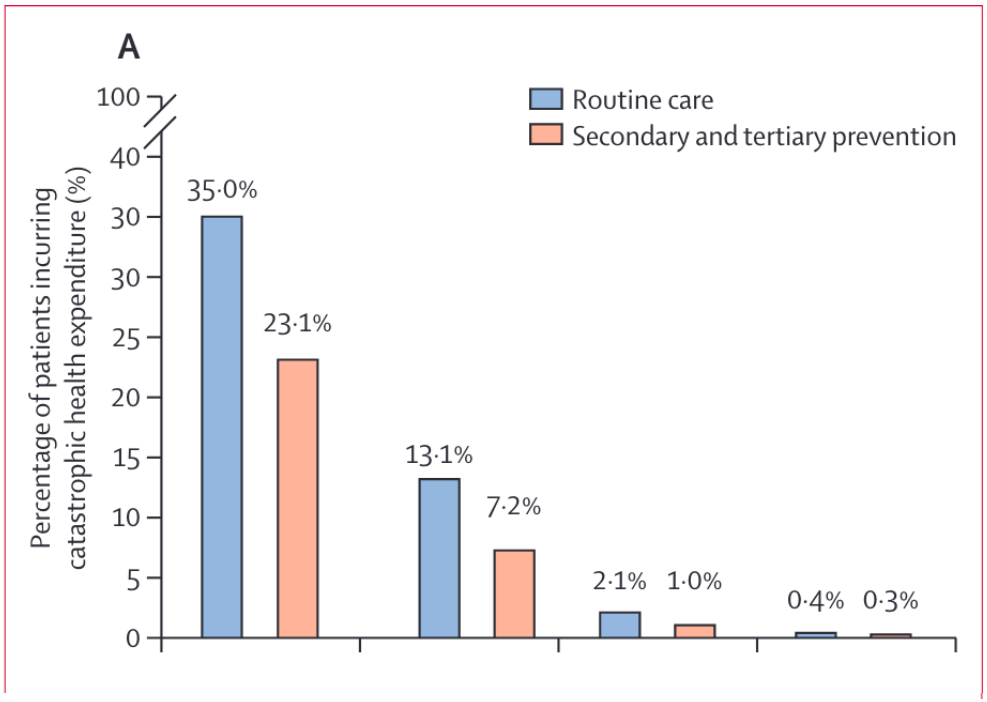


Figure 3: **Number of rheumatic heart disease cases per 1000 population in routine care and combined secondary and tertiary scenarios across different wealth quartiles**

Disaggregated financial outcomes



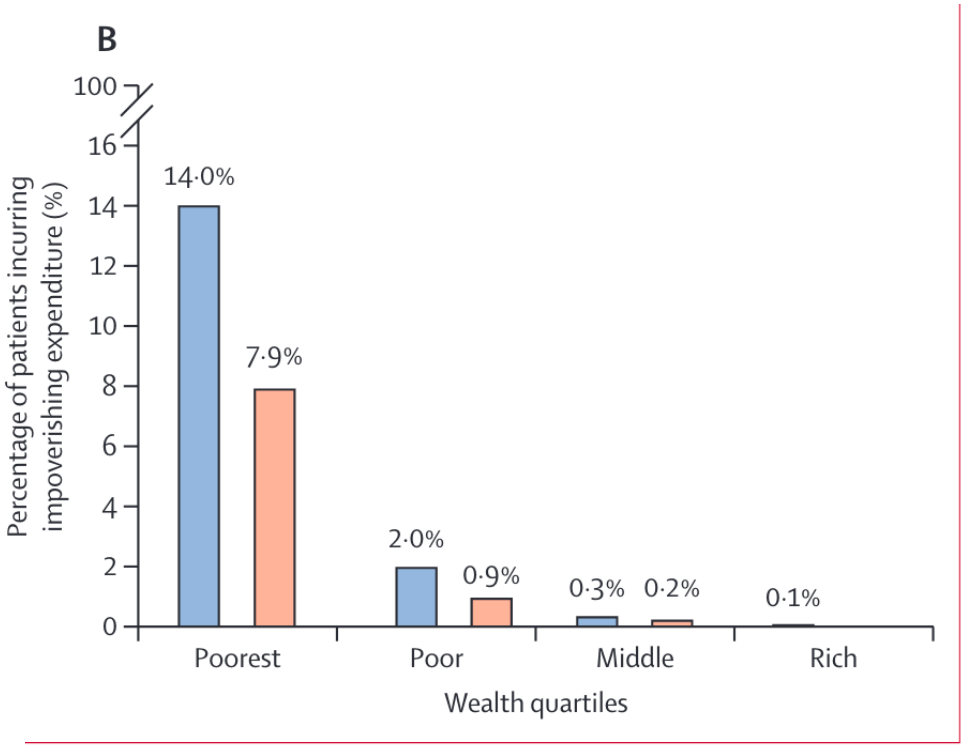


***Figure 4*: Percentage of patients incurring catastrophic (A) and impoverishing (B) health expenditure in the routine care scenario versus the combined secondary and tertiary intervention scenario**

Cost-effectiveness results (total and disaggregated)

	Total	Poorest	Poor	Middle	Rich
Secondary	..	..	..	..	..
Secondary and tertiary	23 050 ($303)	22 902 ($301)	22 596 ($297)	25 663 ($ 338)	32 657 ($428)
Primary, secondary, and tertiary	5 411 599 ($71 205)	2 909 610 ($38 284)	3 961 345 ($52 123)	5 523 536 ($72 678)	8 920 477 ($117 375)
Routine care	D	D	D	D	D
Tertiary	D	D	D	D	D
Primary	D	D	D	D	D
Primary and tertiary	D	D	D	D	D
Primary and secondary	ED	ED	ED	ED	ED

Dominated intervention is defined as an intervention with higher costs and fewer health benefits than alternative interventions. This is an undesirable strategy and should not be recommended. Extendedly dominated is defined as an intervention with an incremental cost-effectiveness ratio higher than the incremental cost-effectiveness ratio of the next, more effective, alternative intervention (ie, the given treatment is dominated by the combination of two alternatives and should not be used to calculate appropriate incremental cost-effectiveness ratios). This is also an undesirable strategy. D=dominated strategies. ED=extendedly dominated strategies.

Table 2: **Incremental cost (in ₹ [US$]) per quality-adjusted life-years gained across income quintiles in various strategies for prevention and control of rheumatic fever and rheumatic heart disease in India**

ECEA compared to DCEA

- Used more often in global health settings
- Focuses more on financial risk protection
- Does not try to aggregate over subgroups
  - More of a “dashboard” approach