Cost-Effectiveness Analysis:
Academic curiosity or practical means to get more “bang” for our “buck”?

- R Scott Braithwaite, MD, MSc, FACP

Chief, Section of Value and Effectiveness Research

Division of General Internal Medicine

NYU School of Medicine

Why Cost-Effectiveness Analysis?

- US does not get good value from health
  - Spends 50% more but leaves 17% uninsured with life expectancies 5-6 years too low
- CEA is best way to measure value in health
  - Compares ***incremental benefit*** with ***incremental cost***
    - ***Incremental benefit*** = ↑ benefit beyond next best alternative
    - ***Incremental cost*** = ↑ cost beyond next best alternative
- Increasing value important for
  - Programmatic decisions
  - Health reform

Why Cost-Effectiveness Analysis?

- Value impacts generalizability and scalability
  - Resources used for study intervention may be resources not available for alternative interventions
  - Does study intervention deliver more health benefit than alternative uses?
- Explicit, systematic, “level playing field”

Why Not Cost-Effectiveness Analysis?

- Difficult to synthesize evidence from different cost-effectiveness studies
- Assumptions in cost-effectiveness not transparent
  - An analysis based on a high-quality study may appear to have same certitude as analysis based on expert opinion
- A policy tool in search of U.S. policy “levers”
  - Other countries use it (e.g. Canada, U.K., Australia, Netherlands) but U.S. stakeholders usually don’t

Research to address limitations of cost-effectiveness analysis

- Synthesizing cost-effectiveness evidence
- Making assumptions underlying cost-effectiveness studies more transparent
- Making cost-effectiveness results more actionable for stakeholders
  - Program planning
  - Incentive design

Cost-effectiveness analysis IS:*

The use of formal analysis to assist decision makers in choosing from among competing alternatives, in situations of limited resources.

Cost-effectiveness analysis is NOT:

- Indiscriminate cost-cutting
- Downsizing
- Bean counting
- The one and only answer

Cost-Consequence Space

Incremental Health Effect	Incremental Cost +	Incremental Cost −
+	Evaluate C/E Ratio	Yes
−	No	Evaluate C/E Ratio

The C/E Ratio

$$\frac{\text{Net Increase in Cost}}{\text{Net Increase in Health Effect}}$$

- A measure of "value for money"
- Comparison of competing alternatives
- Comparison across interventions

Cost-effectiveness Primer

- C/E ratios expressed in
  - Cost per life-year
  - Cost per QALY (quality-adjusted life-year)
- Higher numbers are less favorable
  - $1,000,000/QALY means society would pay one million dollars for an additional year of life
- Lower numbers are more favorable
  - $100/QALY means society would pay only one hundred dollars for an additional year of life

Cost-effectiveness Analysis Primer

- Analyses require evidence about benefit and cost
- Evidence may come from one study but often from mix of different studies and opinion
- ***Sensitivity Analyses*** evaluate impact of uncertainty about evidence
  - Smaller study = larger 95% CI = more uncertainty

Research to address limitations of cost-effectiveness analysis

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  - Program planning
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Objective

- **Research objective**
  - Synthesize cost-effectiveness evidence to identify high-value cardiovascular health services
- **Decision-centered goal:**
  - **Inform consumers to**
    - Engage in more informed health discussions
    - Seek health plans offering high-value services without financial or administrative barriers

Methods – evidence synthesis

- Guiding principles
  - Define “services” broadly
    - Prevention
    - Diagnosis
    - Treatment
    - Management.
  - Limit group using standards of
    - Quality of CEA study
    - Non-obsolescence
    - Consistency of evidence
    - Relevance to consumers

Identifying CV services

- Query National Registry of Cost-Effectiveness Analysis (CEA) Results
  - Summarizes and reviews published, original, English-language cost-utility analysis articles
  - Search for “cardiovascular services” in “U.S.”
- Supplementary searches
  - Published literature
  - Guidelines of national medical and scientific societies
    - Published in peer-reviewed scientific journals
    - Used explicit standardized evidence syntheses.

Definition of “value”

- Value = additional benefit per additional cost
  - “Bang for buck”
  - Inverse of incremental cost-effectiveness ratio
  - Not the same as cost-saving
- High-value = Value better than what we know US willing to pay for
  - ≥ $100,000 per year of good-quality life (QALY)
- Low-value = Value worse than what we know US not willing to pay for
  - ≤ $300,000 per QALY

Braithwaite RS et al, Medical Care, 2008

Quality of CEA Study

- Obtain original publications
- Apply the *Quality of Health Economic Studies*
  - Validated instrument for measuring the quality of cost-effectiveness analyses.
    - 75 is a commonly-used cutoff for high quality
  - Each study was reviewed by at least one reviewer
    - Reviewers had inter-rater kappa of 0.68

Non-obsolescence

- Recommendation by up-do-date guideline(s) of clinical or scientific society.
  - Search National Guideline Clearinghouse
- Only considered guideline if
  - Published in peer-reviewed journals
  - Used explicit evidence rating scales
- High-value service must receive
  - Highest grade of supporting evidence
    - e.g. AHA “Level A”
  - Strongest recommendation in favor
    - e.g. AHA “Level 1”

Consistency of Results

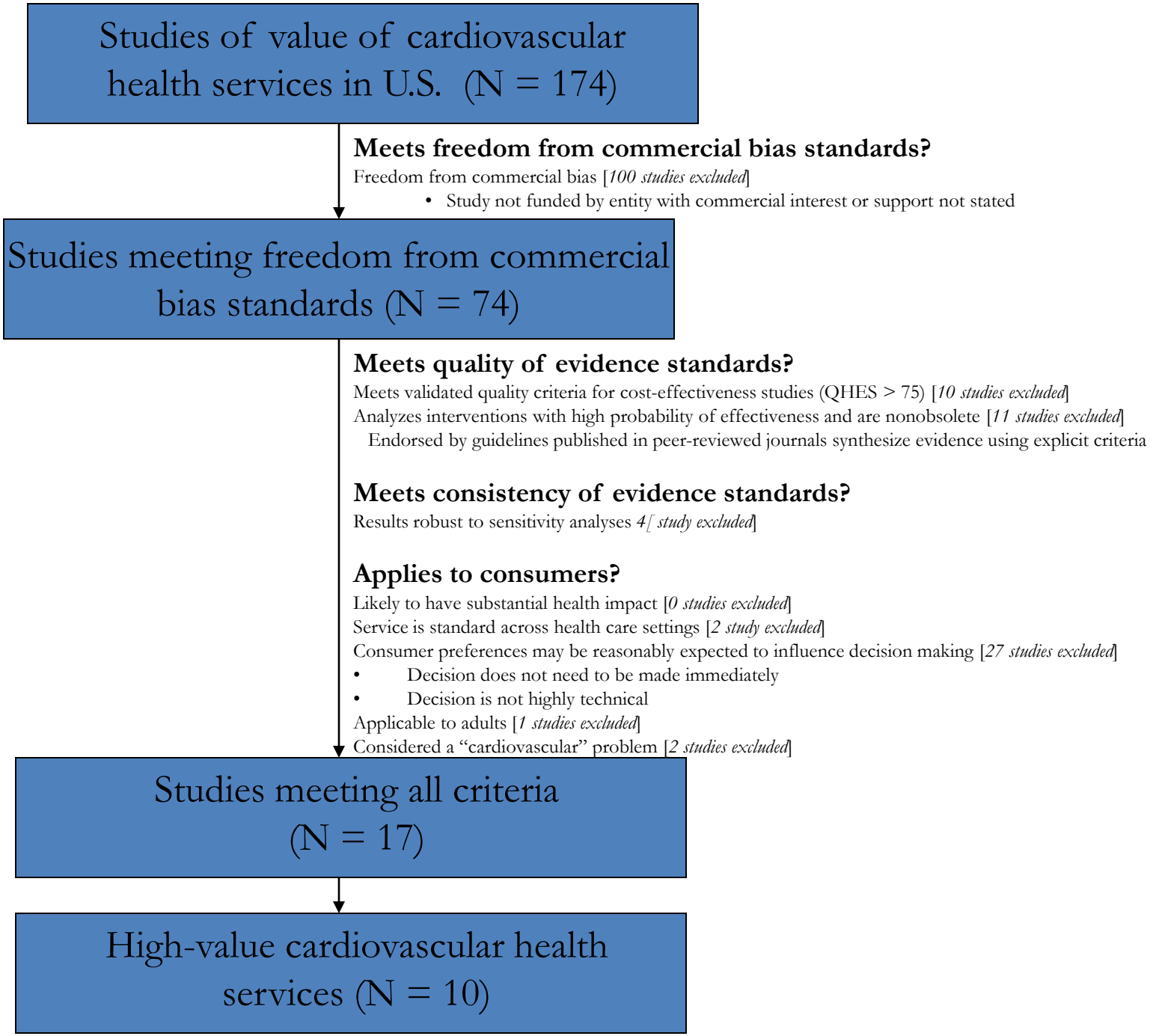
- Results should be consistent if >1 published study addresses the same question
- Results should be robust to alternative but plausible assumptions.
- Consistency assessed by implication for decision making
  - Studies with ICERs of $40k and $70k would be consistent because results are on the same side of decision threshold (<$100k)
  - Therefore both would yield the favorable inference for decision making

Assure consumer relevance

- Problem is not rare
- Service could be standardized across different settings
- Topic would be considered within “cardiovascular” domain by lay audiences.
- Consumer preferences may be reasonably expected to influence decision making
  - Excluded if decision
    - Would need to be made immediately
    - Was too technical to for shared decision making

Sensitivity analyses

- Some payers and consumer groups do not regard quality assessment as sufficiently sensitive for excluding conflict-of-interest bias
  - Base case excludes industry-funded studies
- Sensitivity analysis included industry-funded studies if endorsed by high-grade evidence/guideline
  - United States Preventive Services Task Force


Studies of value of cardiovascular health services in U.S. (N = 174)
Meets freedom from commercial bias standards?
Freedom from commercial bias [100 studies excluded]
• Study not funded by entity with commercial interest or support not stated
Studies meeting freedom from commercial bias standards (N = 74)
Meets quality of evidence standards?
Meets validated quality criteria for cost-effectiveness studies (QHES > 75) [10 studies excluded]
Analyzes interventions with high probability of effectiveness and are nonobsolete [11 studies excluded]
Endorsed by guidelines published in peer-reviewed journals synthesize evidence using explicit criteria
Meets consistency of evidence standards?
Results robust to sensitivity analyses 4[study excluded]
Applies to consumers?
Likely to have substantial health impact [0 studies excluded]
Service is standard across health care settings [2 study excluded]
Consumer preferences may be reasonably expected to influence decision making [27 studies excluded]
• Decision does not need to be made immediately
• Decision is not highly technical
Applicable to adults [1 studies excluded]
Considered a "cardiovascular" problem [2 studies excluded]
Studies meeting all criteria (N = 17)
High-value cardiovascular health services (N = 10)

Results: High-value CV services

High-value health service	Goal of service	People for whom value is likely high
Cholesterol-lowering medication (statin)	Prevent heart attacks	Known coronary heart disease
Cholesterol-lowering medication (statin)	Prevent heart attacks	LDL > 130 and 10-year CHD risk > 5%
Screening for high blood pressure and tx with diuretic, b-bl or ACE	Prevent heart attacks and strokes	Known hypertension
Oral blood-thinning medication (warfarin)	Prevent blood clot to lung	First blood clot without known reason
Injectable blood-thinning medication (low molec-weight heparin)	Prevent blood clot to lung	Just diagnosed with blood clot

Mentor SM and Braithwaite RS, Am J Managed Care, 2011

Results: High-value CV services

High-value health service	Goal of service	People for whom value is likely high
Implantable cardiac defibrillator	Prevent "sudden death"	Congestive heart failure because of a heart attack (EF < 30%)
Blood-thinning medication (aspirin)	Prevent future heart attacks	Coronary heart disease
Anti-platelet medication (clopidogrel) for 12 months	Prevent future heart attacks	After heart attack or other acute coronary events
Medication that reduces strain on heart (beta-blocker)	Prevent future heart attacks	Coronary heart disease
Oral blood-thinning medication (warfarin) and necessary labs	Prevent future strokes	Non-valvular atrial fibrillation and >=1 stroke risk factors

Mentor SM and Braithwaite RS, Am J Managed Care, 2011

Sensitivity Analyses

Industry funded studies with strong support of guidelines (N = 10)

Meets quality of evidence standards?

Meets validated quality criteria for cost-effectiveness studies (QHES > 75) [*1 studies excluded*]

Analyzes interventions with high probability of effectiveness and are nonobsolete [*0 studies excluded*]

Endorsed by guidelines published in peer-reviewed journals synthesize evidence using explicit criteria

Meets consistency of evidence standards?

Results robust to sensitivity analyses and consistently suggest ICER < $100,000 per LY or per QALY [*3 studies excluded*]

Applies to consumers?

Likely to have substantial health impact [*0 studies excluded*]

Service is standard across health care settings [*0 study excluded*]

Consumer preferences may be reasonably expected to influence decision making [*0 studies excluded*]

- Decision does not need to be made immediately
- Decision is not highly technical

Applicable to adults [*0 studies excluded*]

Considered a "cardiovascular" problem [*0 studies excluded*]

Studies meeting all criteria (N = 7)

High-value cardiovascular health services (N = 3)

Sensitivity Analyses: High-value CV services

High-value cardiovascular health service	Goal of service	People for whom value is likely high
Smoking cessation counseling, NRT, and pharmacotherapy	Stop smoking	All smokers
Stroke prevention with anti-platelet drugs (aspiring or clopidogrel)	Prevent stroke	People who have had a stroke or TIA (“mini-stroke”)
Aspirin for primary prevention of cardiovascular in men	Prevent heart attacks	Middle-age men with 10 year CHD risk >5% without increased bleeding risk

Limitations

- Does not evaluate an exhaustive list of cardiovascular services
- Very few services have been studied in all relevant patient groups.
- Relatively sparse results highlight the importance of expanding comparative effectiveness research

Conclusions

- Many common cardiovascular services are identifiable as high-value
  - Expensive as well as cheap
- Identifying high-value services may help
  - Engage in more informed health discussions
  - Seek health plans that offer services without financial or administrative barriers
- Approach can be applied to other services

Research to address limitations of cost-effectiveness analysis

- Synthesizing cost-effectiveness evidence
- Making assumptions underlying cost-effectiveness studies more transparent
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  - Program planning
  - Incentive design

Objective

- Research Objective
  - Can we augment standard cost-effectiveness analysis methods to develop a sensitivity analysis based on quality of evidence?
- Decision centered goal
  - Clinicians and policy-makers often wonder “what goes into the model”
  - Make the uncertainty from low-quality evidence transparent to model users so they know whether to “trust the model” for their particular question

Methods

- Basic concept of our approach
  - When potential information sources have insufficient quality of evidence, don't use them
  - Instead, assume that little is known by using uninformative distributions over wide range
  - Don't obscure questionable data under a "false veneer of mathematical certitude"
    - **Warning**! If you set evidence standards very high, not much of the available evidence may qualify.

Methods

- Assess quality of evidence using USPSTF guidelines
  - ***Study design***
    - Design differs from controlled experiment
  - ***Internal validity***
    - Results represent truth in study population
  - ***External validity***
    - Results represent truth in target population
- Our approach can be used with ***any*** evidence-evaluation hierarchy
  - We chose USPSTF guidelines because of ubiquity not because of rigor

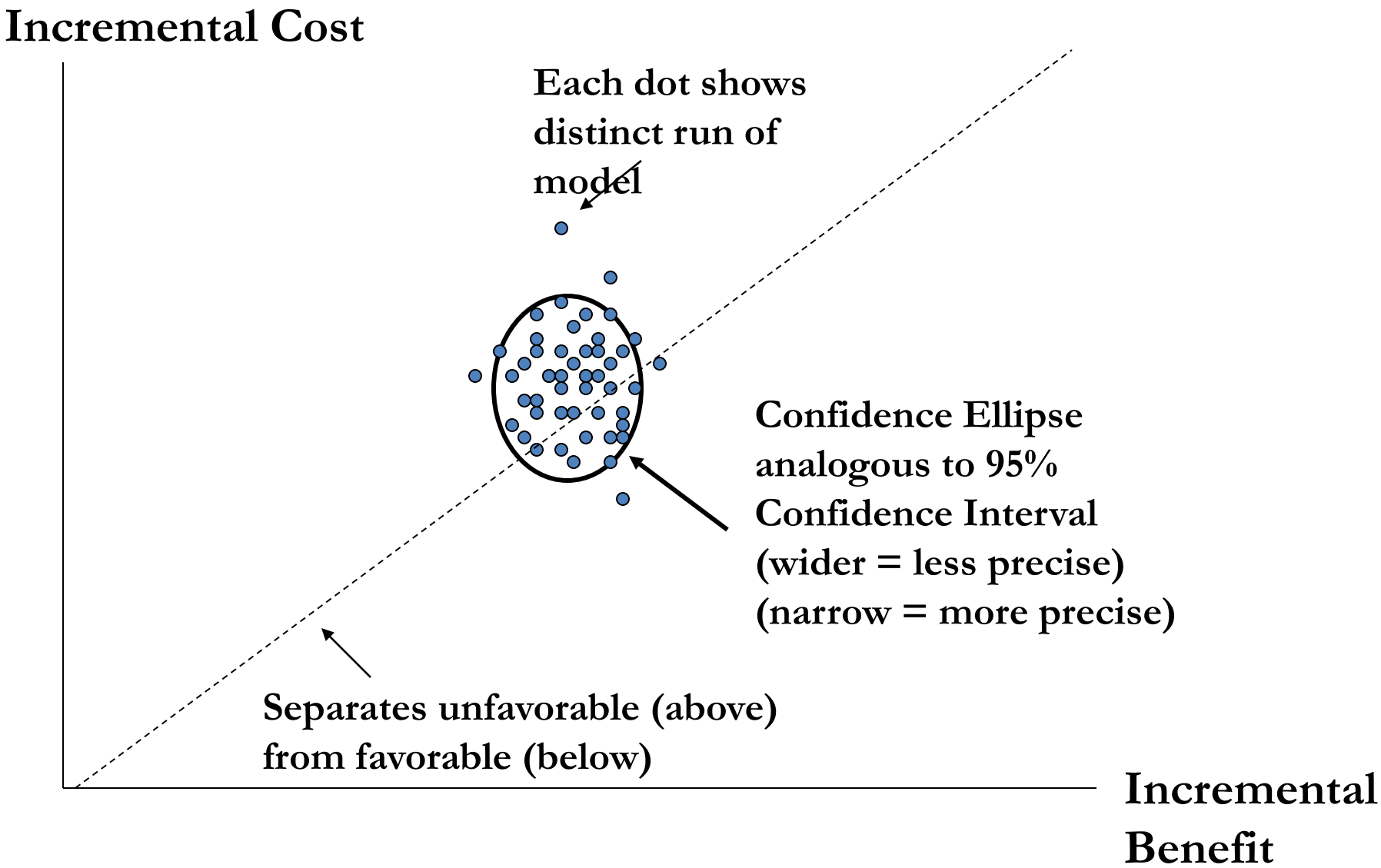
Methods

- Set minimum standard in each evidence domain
  - These can be “dialed” up or down at will
- Evaluate each possible source of evidence
- If source meets evidence criterion, use its 95% confidence interval in the analysis
- If evidence does not meet criterion, do not use it in the analysis
  - Instead use uninformative (“wide”) distribution

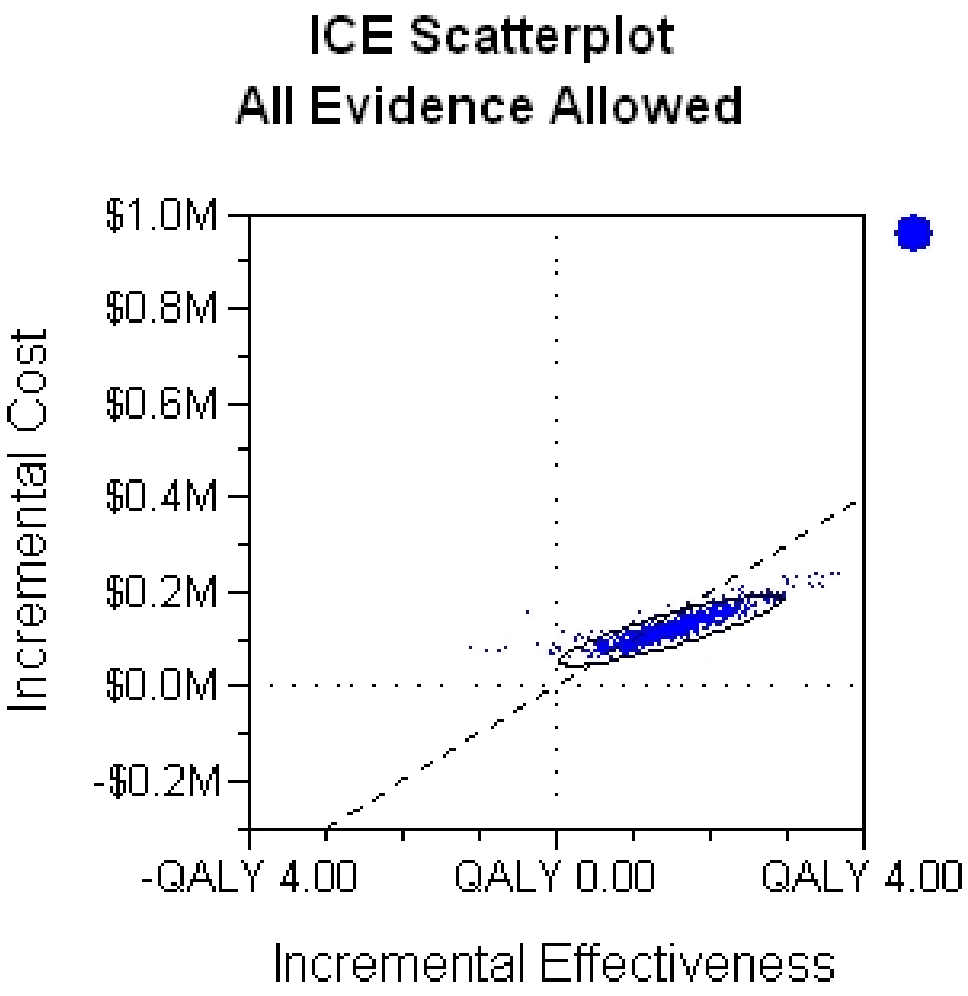
Test Case: Directly observed therapy for HIV antiretrovirals

- Base Case: No evidence criteria
  - All 17 data sources eligible for parameter estimation
- ***Study Design*** set to highest standard ("1")
  - ***13*** out of 17 sources were eligible
- ***Internal Validity*** set to highest standard ("good")
  - ***9*** out of 17 sources were eligible
- ***External Validity*** set to highest standard ("high")
  - ***5*** out of 17 sources were eligible
- ***All three criteria*** set to highest standards
  - ***3*** out of 17 sources were eligible

Cost-Effectiveness Plane Primer

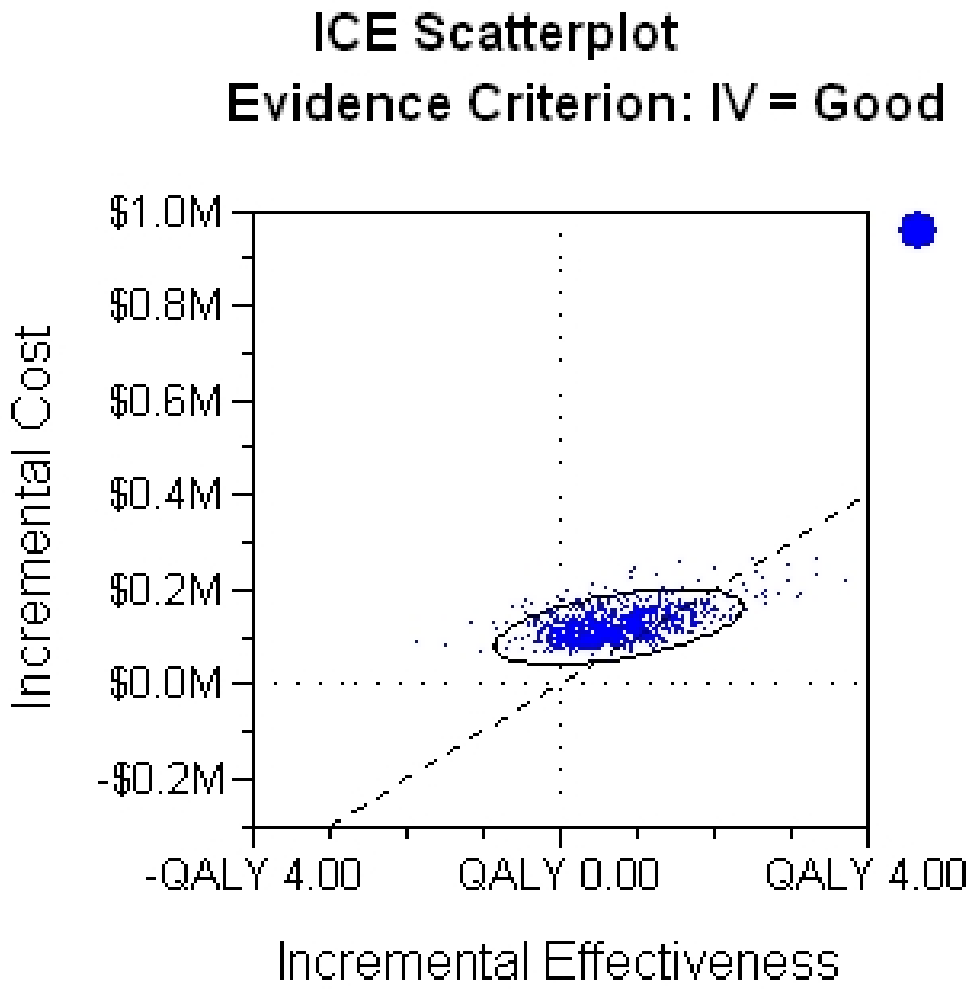


Results: All Evidence



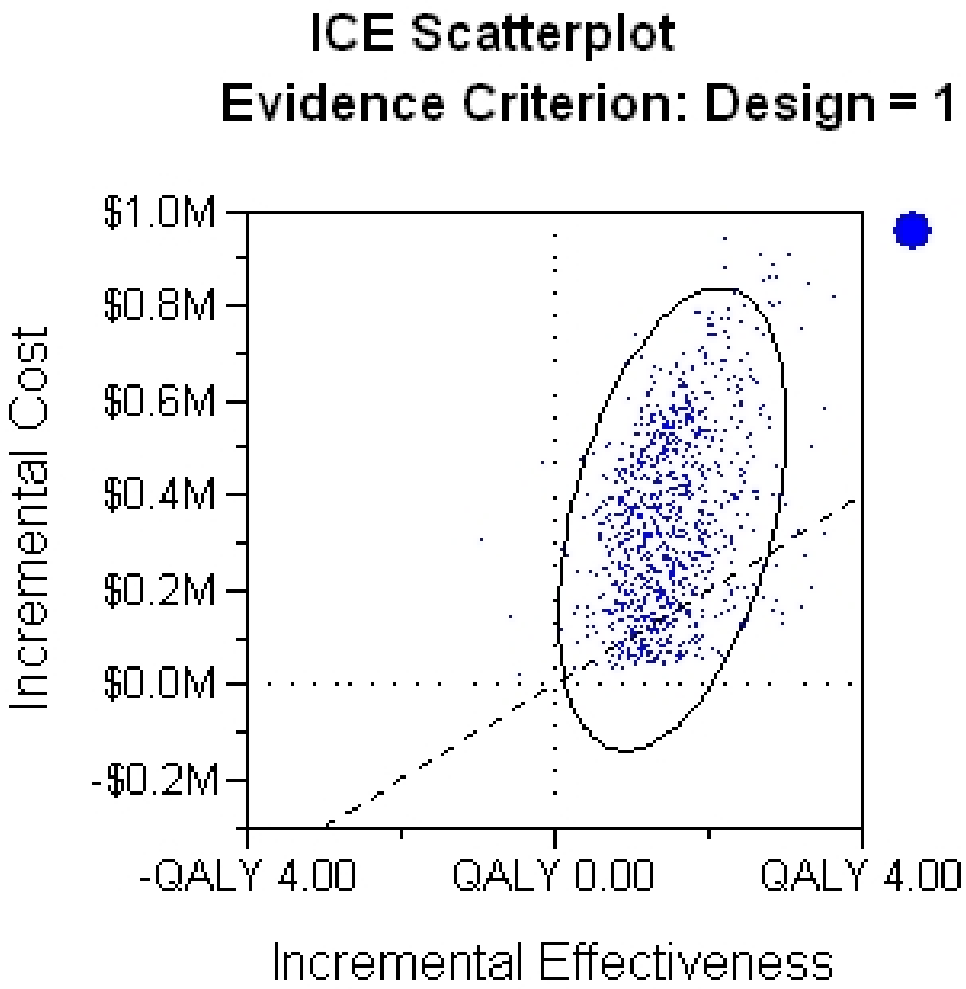
Braithwaite RS et al, Ann Intern Med 2007; 146:133-141

Results: Internal Validity "Good"



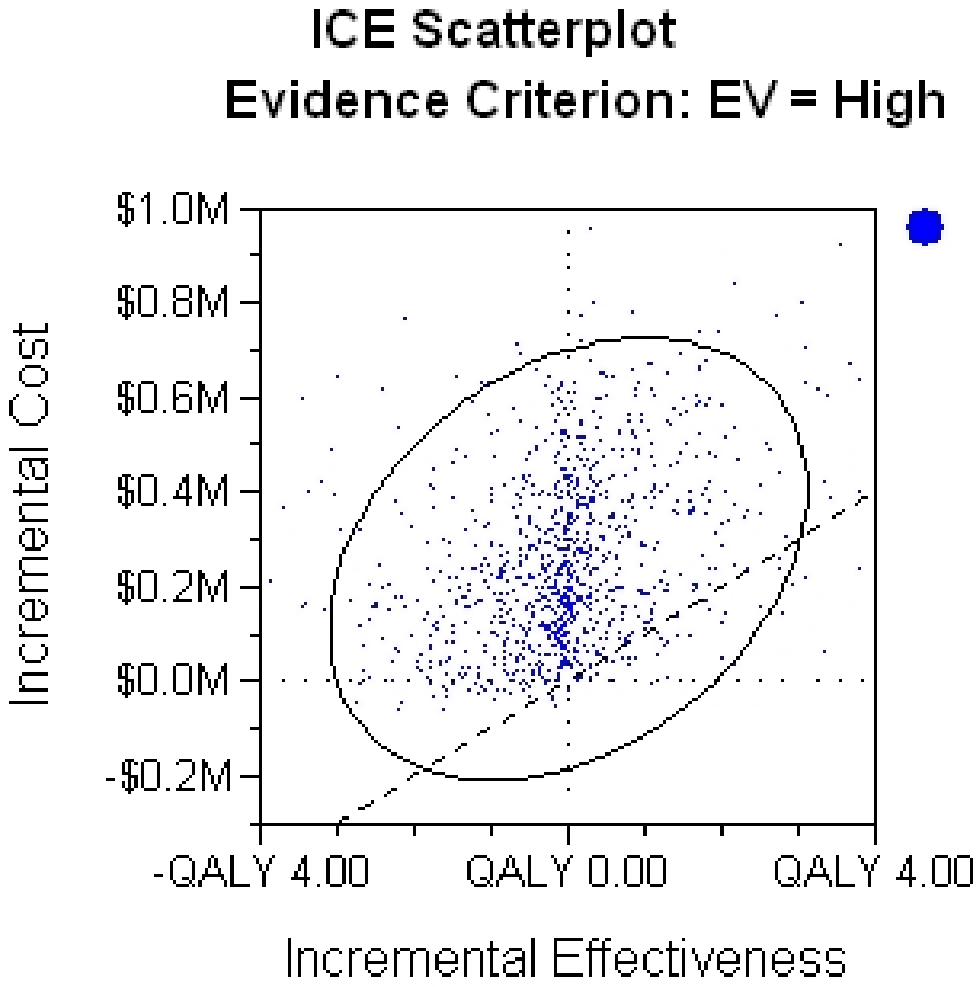
Braithwaite RS et al, Ann Intern Med 2007; 146:133-141

Results: Study Design "1"



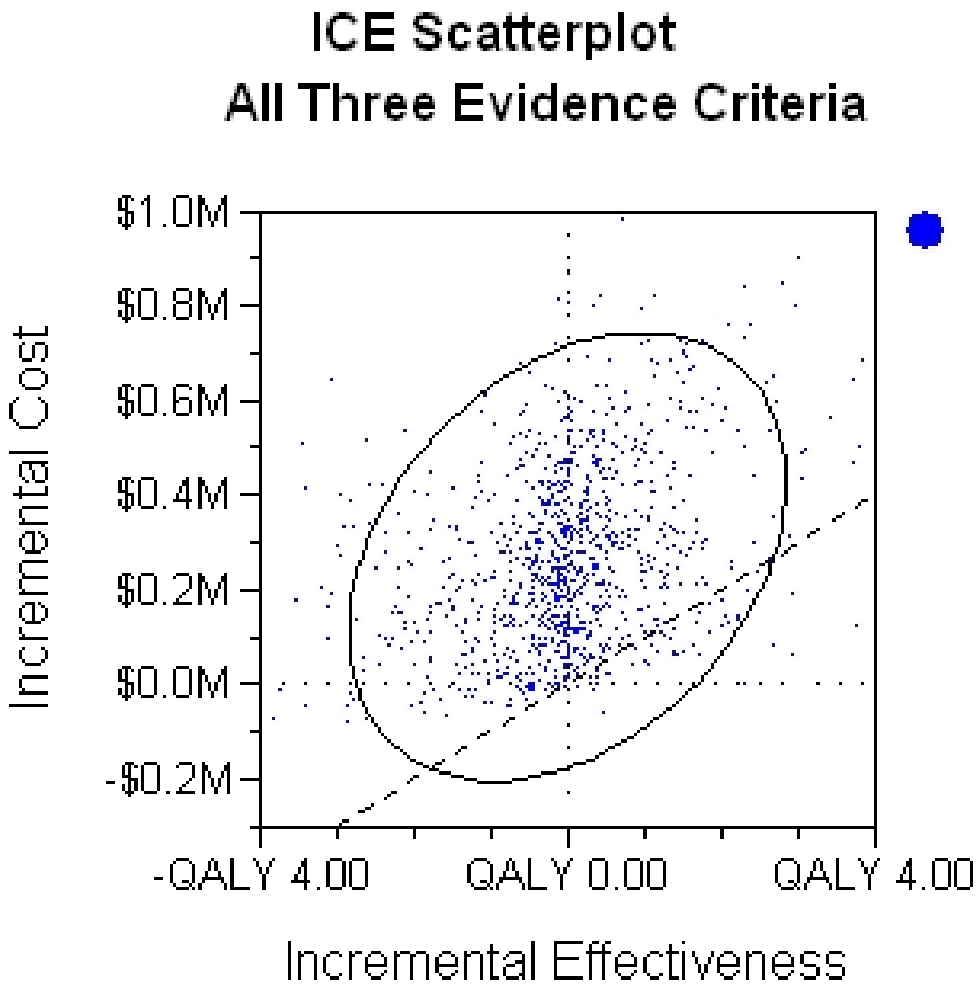
Braithwaite RS et al, Ann Intern Med 2007; 146:133-141

Results: External Validity "High"



Braithwaite RS et al, Ann Intern Med 2007; 146:133-141

Results: All 3 Criteria



Braithwaite RS et al, Ann Intern Med 2007; 146:133-141

Conclusions

- Quality of evidence may have profound impact on the precision and estimates of CEAs
- Stricter evidence criteria may produce more uncertain results because there are fewer studies to base assumptions on
- Approach shows when evidence is not good enough for decision making
  - Need higher-quality information on HIV DOT

Research to address limitations of cost-effectiveness analysis

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Background

- Cost-sharing becoming a standard “volume knob” to control utilization in U.S.
- Cost-sharing has
  - Great potential to control costs
  - Great potential to cause harm
- Increasing calls to link cost-sharing to value (e.g. value-based insurance design)
  - No cost-sharing for high-value services
  - Same or increased cost-sharing for low-value services

Purpose

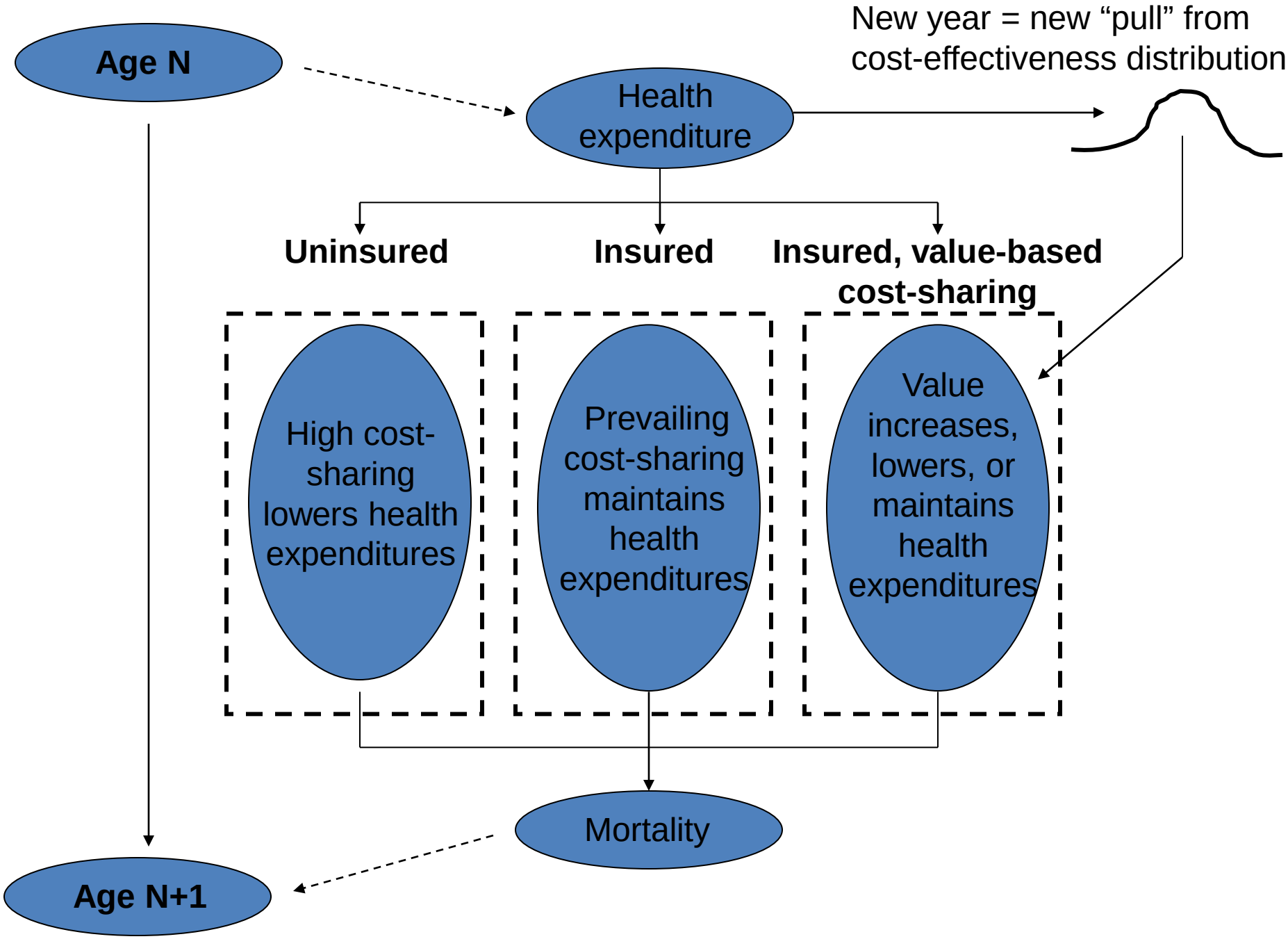
- Research Objective
  - To estimate the impact of value-linked cost-sharing if it were applied systematically across US health system
- Decision-centered Goal
  - To determine whether value-based insurance design should be scaled up or rolled back

Methods

- From RAND, we can estimate the impact of cost-sharing amount on health service demand

Cost-sharing	0%	18% (Current)	30%	100%
Relative utilization	1.06	1.00	0.85	0.65

- Results confirmed by >100 observational studies

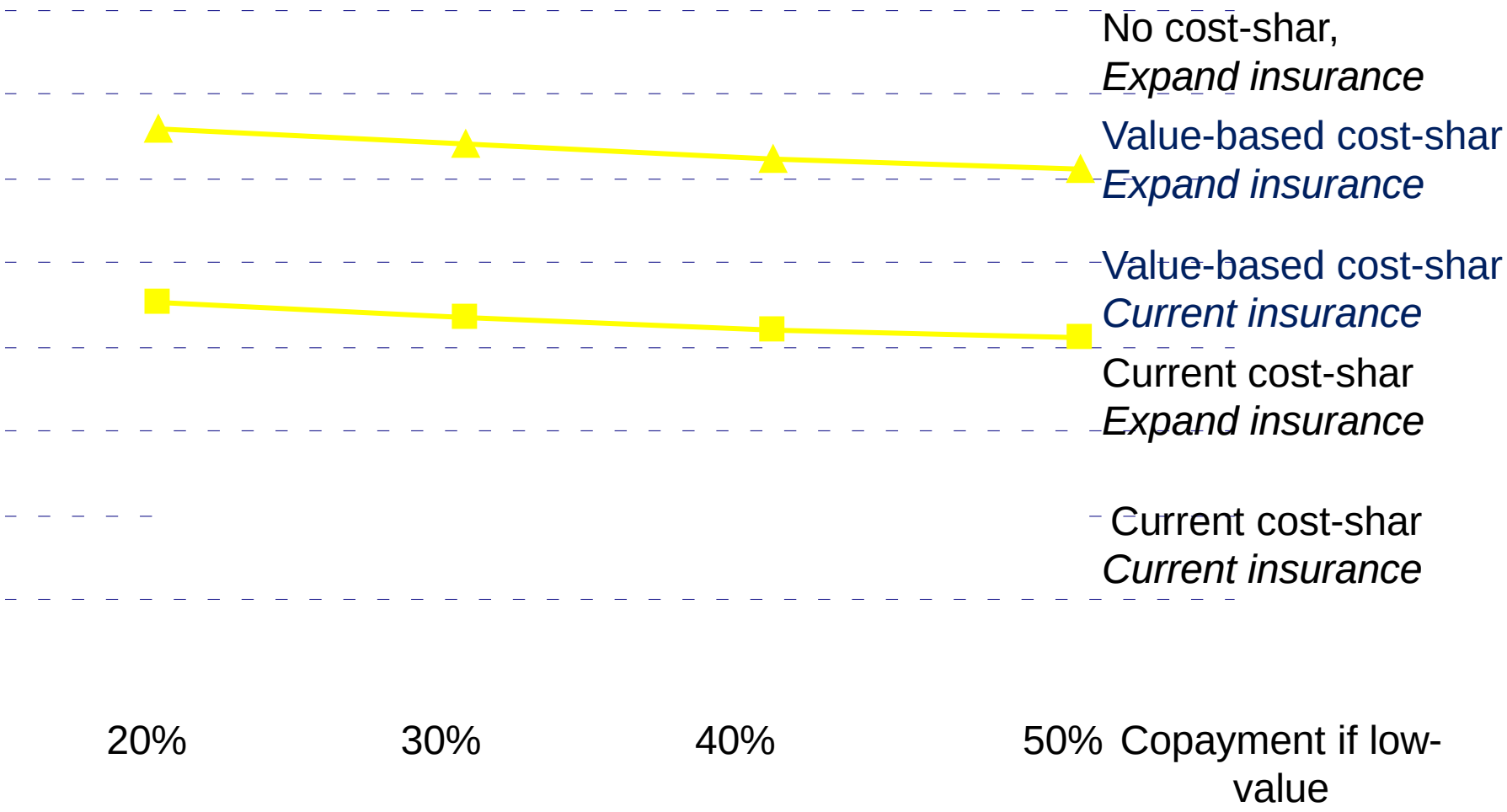

New year = new "pull" from cost-effectiveness distribution
Age N
Health expenditure
Uninsured
Insured
Insured, value-based cost-sharing
High cost-sharing lowers health expenditures
Prevailing cost-sharing maintains health expenditures
Value increases, lowers, or maintains health expenditures
Mortality
Age N+1

Methods

- Possible way to link cost-effectiveness results to cost-sharing
  - > $300K/QALY: ***Increase*** cost-sharing
  - $100K-300K/QALY: ***No Δ*** cost-sharing
  - < $100K/QALY: ***Waive*** cost-sharing
  - Cost-saving: ***Reverse*** cost-sharing?

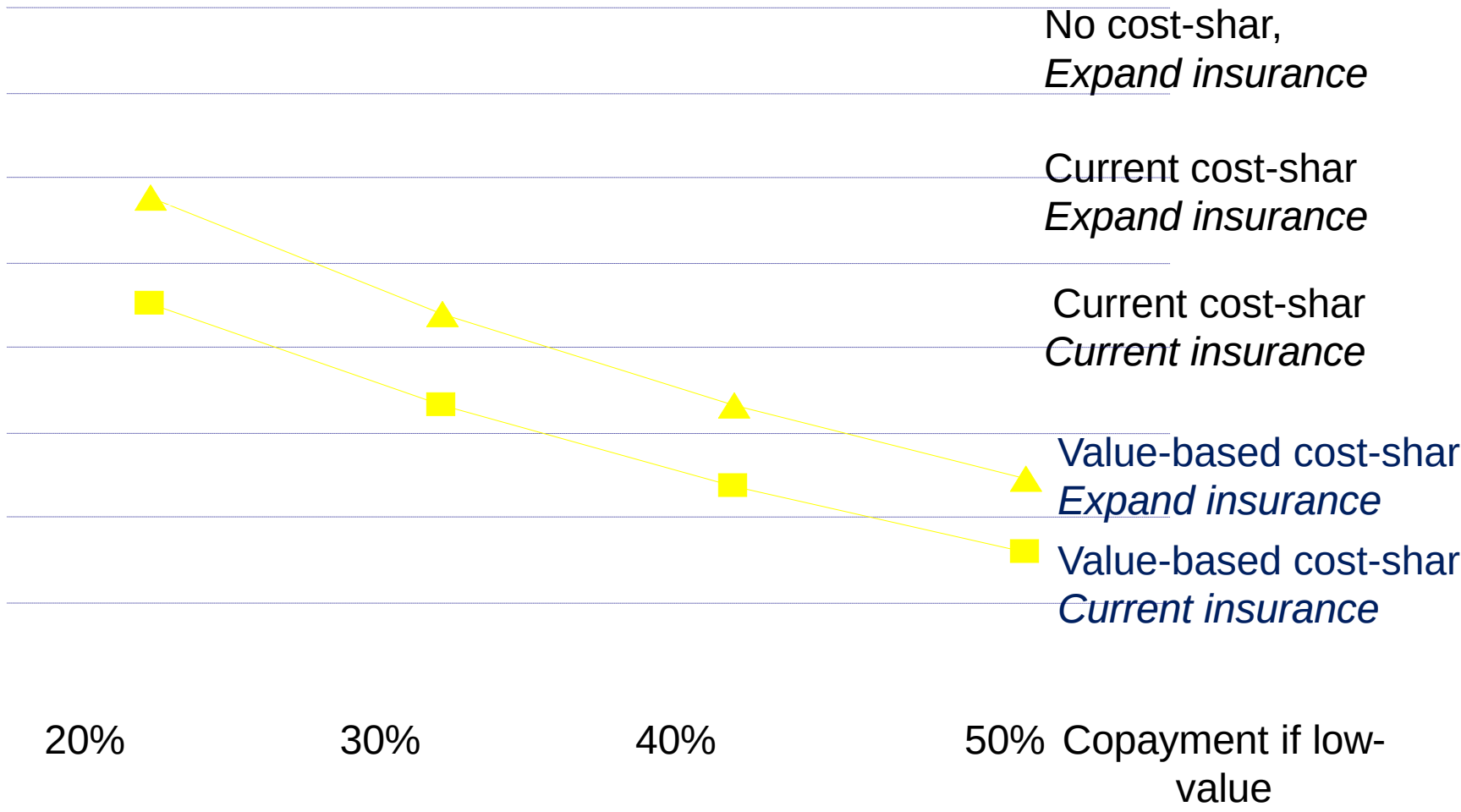
Braithwaite RS et al, Ann Intern Med. 2007

Life Expectancy Gain (Years)



Braithwaite RS et al, PLoS Medicine, 2010

Annual per-capita cost (2003 $)



Braithwaite RS et al, PLoS Medicine, 2010

Conclusions

- Value-linked cost-sharing may increase life expectancy from health care while reducing costs
- Costs may be lowered sufficiently to offset incremental expenditures from expanding health insurance coverage

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Modeling HIV Prevention Priorities in New York City

- Research Objective
- Construct an operations research model to assist HIV prevention planners determine the optimal combination of prevention strategies that will maximize the number of new infections averted, within funding limits.
- Decision-centered goal:
- How much additional money to ask for, and what to spend it on?

Modeling HIV Prevention Priorities in New York City

- **Model Features:**
  - Includes 11 required and 8 recommended, or 19/24 interventions/strategies outlined by CDC in ECHPP
  - Allows decision maker to prioritize HIV prevention strategies based upon strength of evidence, budget, and feasibility constraints.
  - Explores the interplay between various HIV prevention strategies.
  - Accounts for complex behavioral heterogeneity (social network characteristics, partner mixing, etc.)
  - Includes behavioral feedback loops that may influence transmission risk.

Limitations

- Preliminary results
  - Additional sensitivity analyses needed
  - Some inputs need further verification
  - Formal optimization of portfolio in progress
- Evidence limitations
- Only considers the ECHPP interventions
  - Future work to consider broader range of interventions and targets

Conclusions

- Potential to reduce HIV incidence by 60%
  - Focus on HIV-infected
  - What is effective may not be low cost/infection averted
- Future work
  - Develop and evaluate new targeting for high-value interventions
  - Virtuous cycle for public health/academic partnership
    - Use inferences from models to inform decisions
    - Evaluate impact of decisions
    - Update model with new data
    - Repeat

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Community-based interventions aimed at HTN and colorectal cancer disparities

- **Intervention #1 (hypertension):** Therapeutic lifestyle change via **M**otivational **Int**erview (MINT or MI), a counseling approach to motivate behavioral change and help people commit to it
- **Intervention #2 (CRC):** Patient Navigation (PN) based on the community health worker (CHW) model: *"…to help patients access and chart a course through the healthcare system"*
- **Intervention #3 (Hypertension and CRC):** MI and PN
- **Where in community?**
  - Mobile vans in front of churches and black-owned barbershops in Central Harlem, NYC (part of ongoing program)

CEA and Community-based research

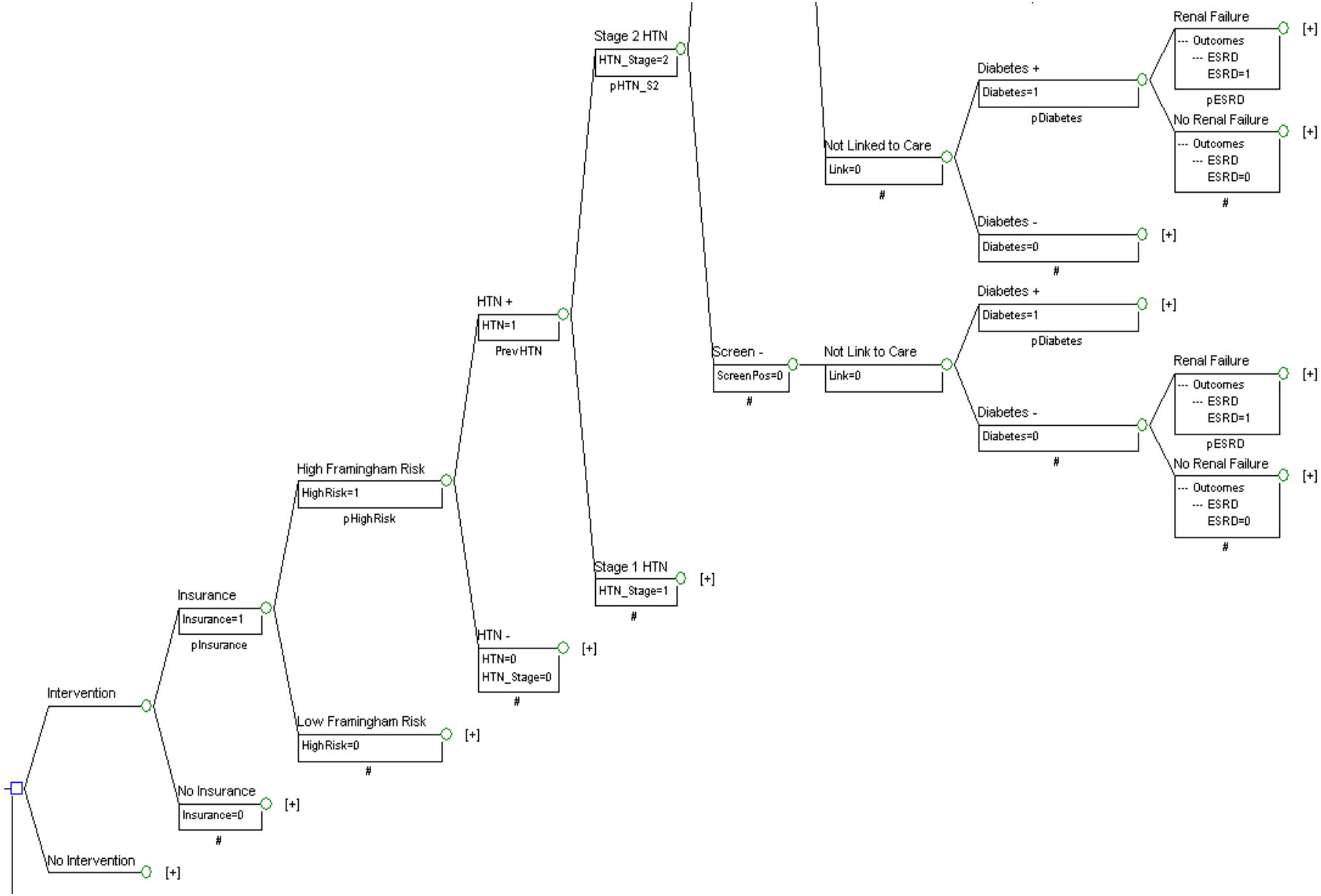
- **Research Objectives**
  - What is cost-effectiveness of these community-based interventions?
  - Is value sufficient for scaling?
  - Will it deliver more “health” for the money compared to alternative uses?

- **Decision Goal:**
  - Should one or both interventions be rolled out, and given funding priority relative to other interventions addressing important health disparities in urban black men?

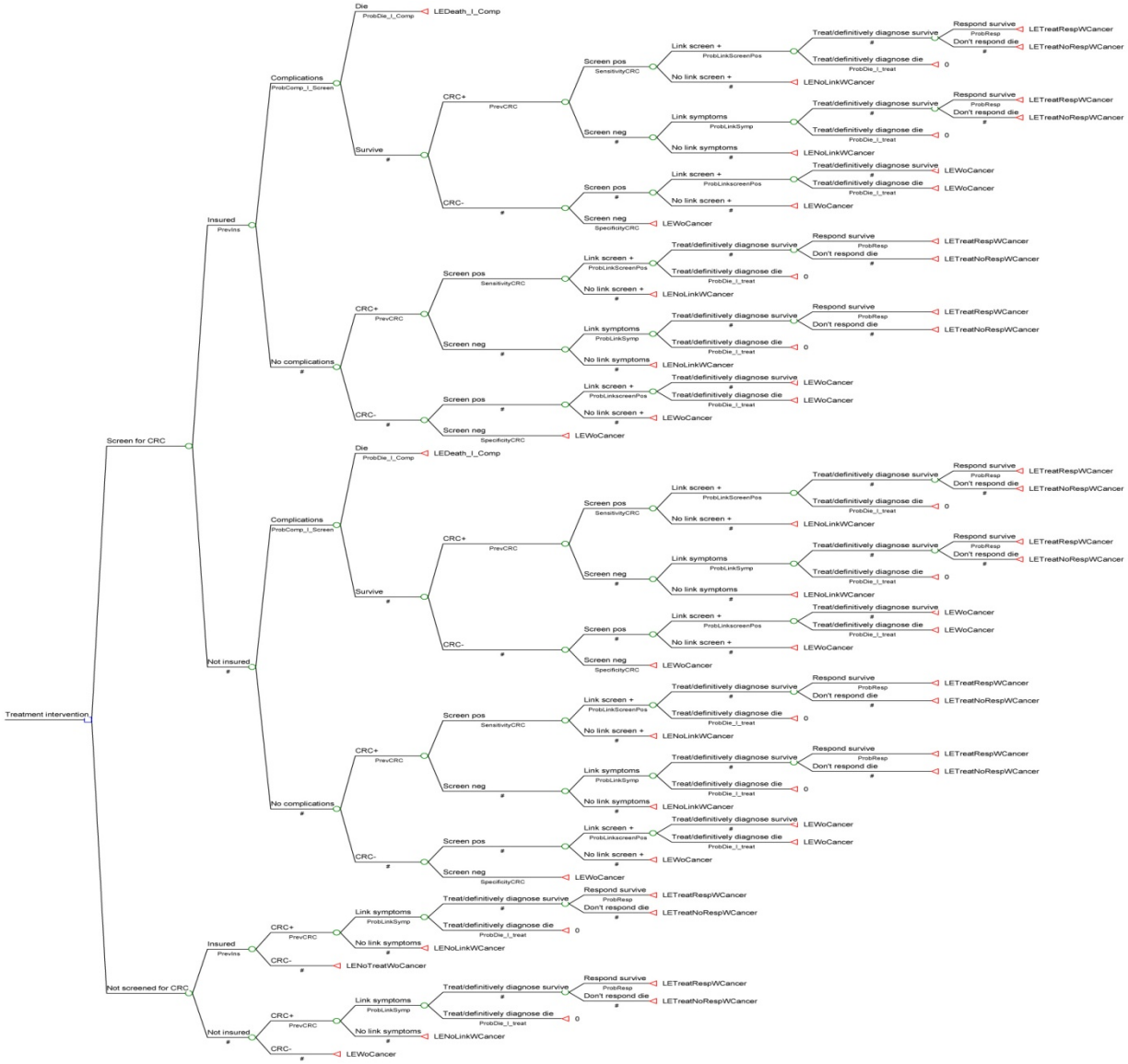
Methods

- Computer simulation for African-American men who reside in NYC incorporating two distinct outcomes:
  - Blood pressure control and the consequences of high BP over a period of time
  - Colorectal cancer screening and the long-term consequences of screening and the disease if left untreated.

Hypertension Model-Part I



Colorectal Cancer Model



Results

- Models were created while data is collected
  - Like “cartridges" into which data can be inserted, once it is collected and analyzed
- After data is inserted:
  - What is long-term effect of these 2 community-based intervention on life expectancy and high-quality life expectancy?
  - What is their value?
    - How much “health” would be bought if they were rolled-out, and how would this compare to the health that could be “bought” by spending the same money on alternative programs?

Research to address limitations of cost-effectiveness analysis

- Synthesizing cost-effectiveness evidence
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Conclusions

- Methods exist to
  - Assess whether sufficient cost-effectiveness evidence could possibly impact a decision
  - Make evidence uncertainty transparent to inform whether cost-effectiveness should impact a decision
- Decision suitable for impact involve
  - Getting the most “health” from a portfolio of interventions by scaling up or down funding
  - Impacting incentives as well as coverage

Questions ??????