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Economics of palliative care

Key concepts and practical considerations

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12th Annual Kathleen Foley Palliative Care Retreat in La Jolla, California

Overview

Part 1: Conceptual issues (May)

- Health economic evaluation: what and why?

- Economic evaluation and palliative care

Part 2: Key issues in the evidence base (Aldridge)

- Dying in America study

- Group presentations of key articles

Part 3: Practical considerations (May)

- Economic evidence on palliative care

- Practical considerations in conducting a study

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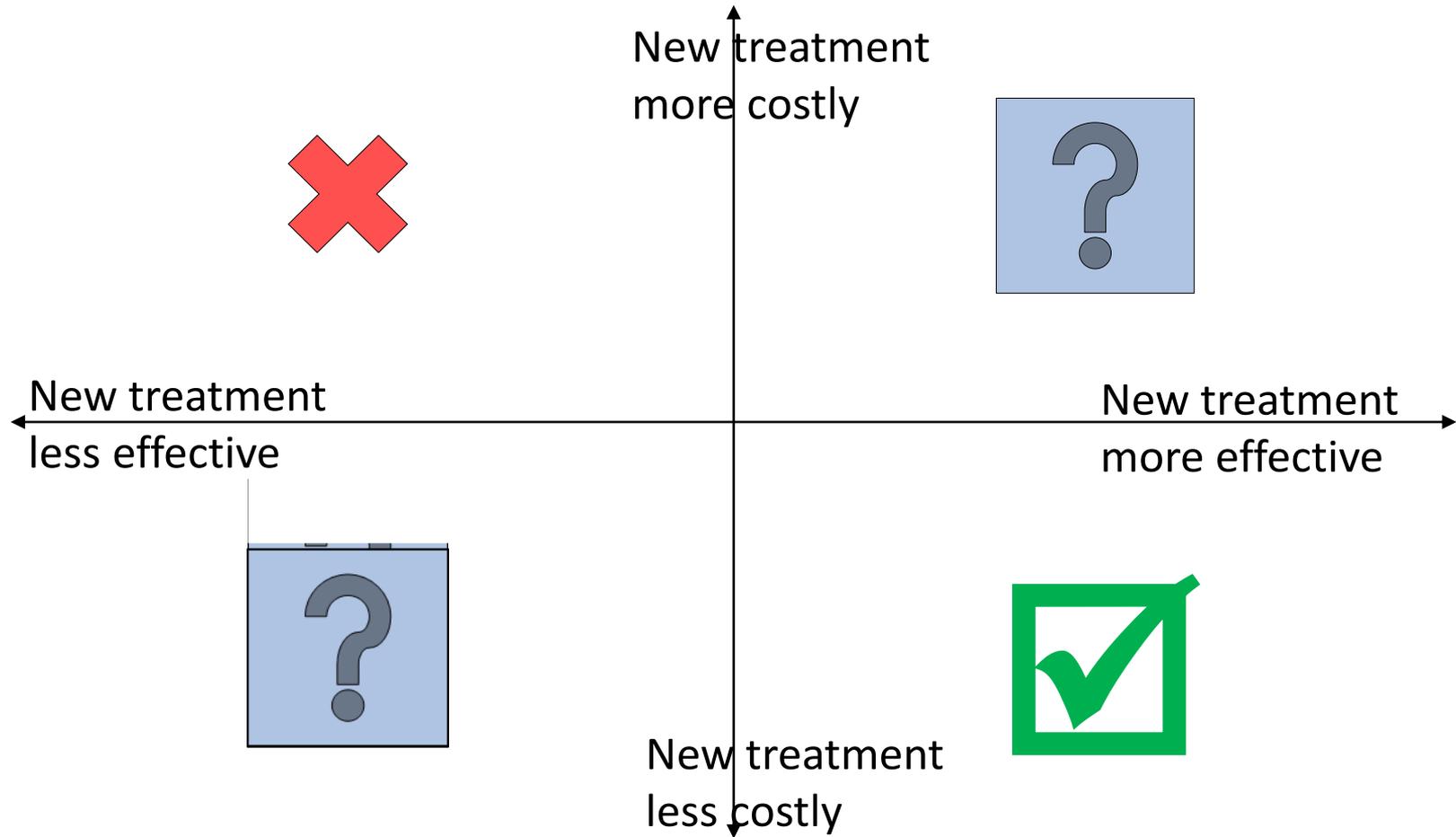# Economic evaluation

What is economic evaluation?

'Full' economic evaluation has two components:

- Measuring treatment effect on costs
  - Formal costs: e.g. hospital, GP, nursing home, out-of-pocket pharma
  - Informal costs: care & help provided by friends, family

- Measuring treatment effect on outcomes
  - Patient outcomes: e.g. survival, HRQoL
  - Family outcomes: e.g. caregiver HRQoL

  ➢ 'Cost-consequence' analysis
    - cost-effectiveness, cost-utility, cost-benefit, etc

Economic evaluation

Cost-consequence analysis

New treatment more costly

New treatment less effective

New treatment more effective

New treatment less costly

Economic evaluation
Why do we do economic evaluation?

- A tool for <u>managing scarcity</u>

  - Unrelated to overall budget or who pays - <u>a fact of life</u>

  - Available resources < Cost of health-related demands

    ➢ Decisions in allocation: what do we pay for?

    ➢ Every decision has an "opportunity cost"

- A tool <u>we each use every day</u>

  - Each of us has finite budgets at work and at home

    ➢ Decisions in allocation and "opportunity cost"

Everyday economic evaluation

Everyday economic evaluation

- Sky subscription was €78 per month…

Everyday economic evaluation

- Sky subscription was €78 per month…

= (78 * 12) = €936 per year…

Everyday economic evaluation

- Sky subscription was €78 per month…
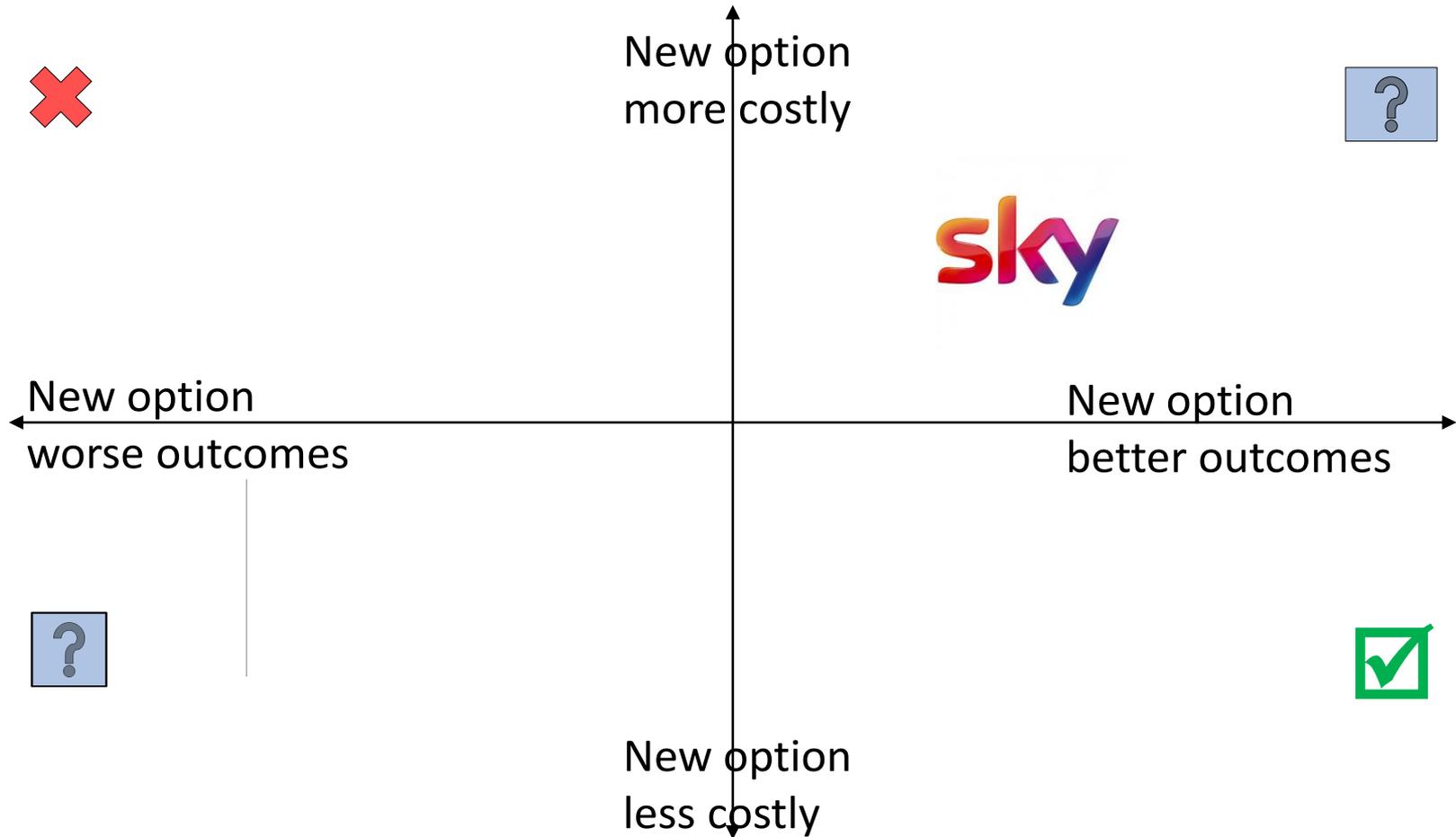
= (78 * 12) = €936 per year…

= (936 * 18) = €16,848

Everyday economic evaluation

- Sky subscription was €78 per month…

= (78 * 12) = €936 per year…

= (936 * 18) = €16,848

- We can choose to spend €16,848 on Sky over the course of our son's childhood

  - And if costs<benefits then it might be the right decision

  - **BUT** that decision has an <u>opportunity cost</u> - this money could instead go on a college fund, dental care, trumpet lessons…

Everyday economic evaluation

Cost-consequence analysis

New option
more costly

New option
worse outcomes

New option
better outcomes

New option
less costly

Everyday economic evaluation

Cost-consequence analysis

New option
more costly

New option
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better outcomes

New option
less costly

Everyday economic evaluation

Cost-consequence analysis

Economic evaluation

Summary

- Economic evaluation is a comparison of different options for their effect on costs and on outcomes

- Our aim is to ensure best care for greatest number of people through wise allocation of resources, which will always be <u>scarce</u> and have <u>alternate uses</u>

- Though abstraction inevitable in practice, principles are familiar & intuitive

- <u>Timeframe</u> is key because unlike many outcome variables costs add up (€78 versus €16,848)

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HE evaluation and palliative care

Two components to economic evaluation:

- Measuring treatment effect on costs

- Measuring treatment effect on outcomes

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HE evaluation and palliative care

Two components to economic evaluation:

- **Measuring treatment effect on costs**

- ~~Measuring treatment effect on outcomes~~

In PC studies, 'consequence' part typically fudged through 'non-inferiority' assumption

HE evaluation and palliative care

The QALY problem

Two components to economic evaluation:

- **Measuring treatment effect on costs**

- ~~Measuring treatment effect on outcomes~~

In PC studies, 'consequence' part typically fudged through 'non-inferiority' assumption

- i.e. that outcomes for intervention group patients are at least no worse than those for comparison group patients

  ➢ Cost analysis (or cost-minimisation analysis)

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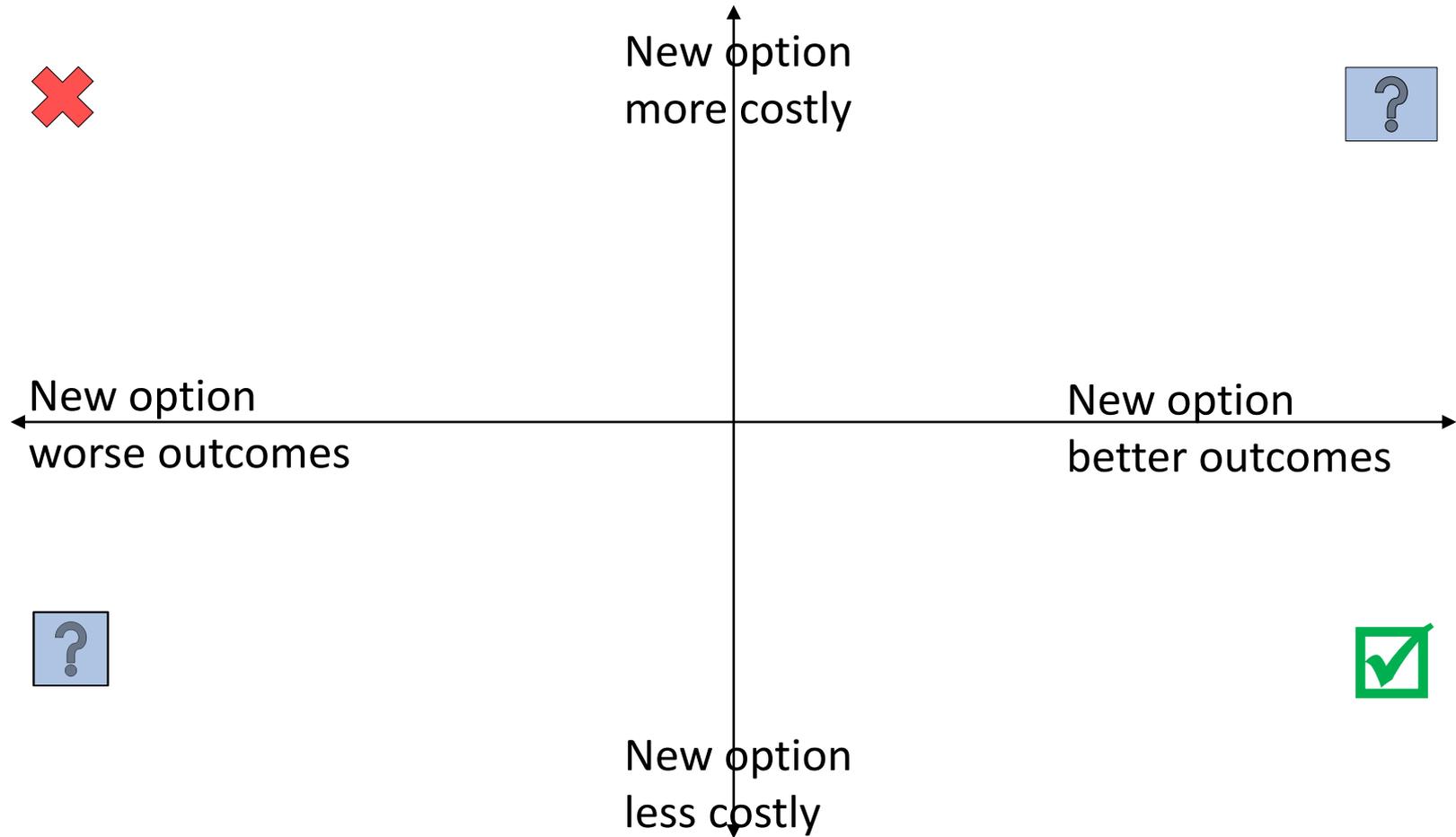
HE evaluation and palliative care

Why is measuring outcomes so difficult?

- Generic issues in EOL research:

  - Sampling, recruitment and retention

  - Which outcomes, tools, perspectives?

- **<u>Comparability</u>**

  - Remember: our aim is to ensure best care for greatest number of people through wise allocation of resources, which will always be <u>scarce</u> and have <u>alternate uses</u>

  - How to compare all health care interventions on one outcome scale?
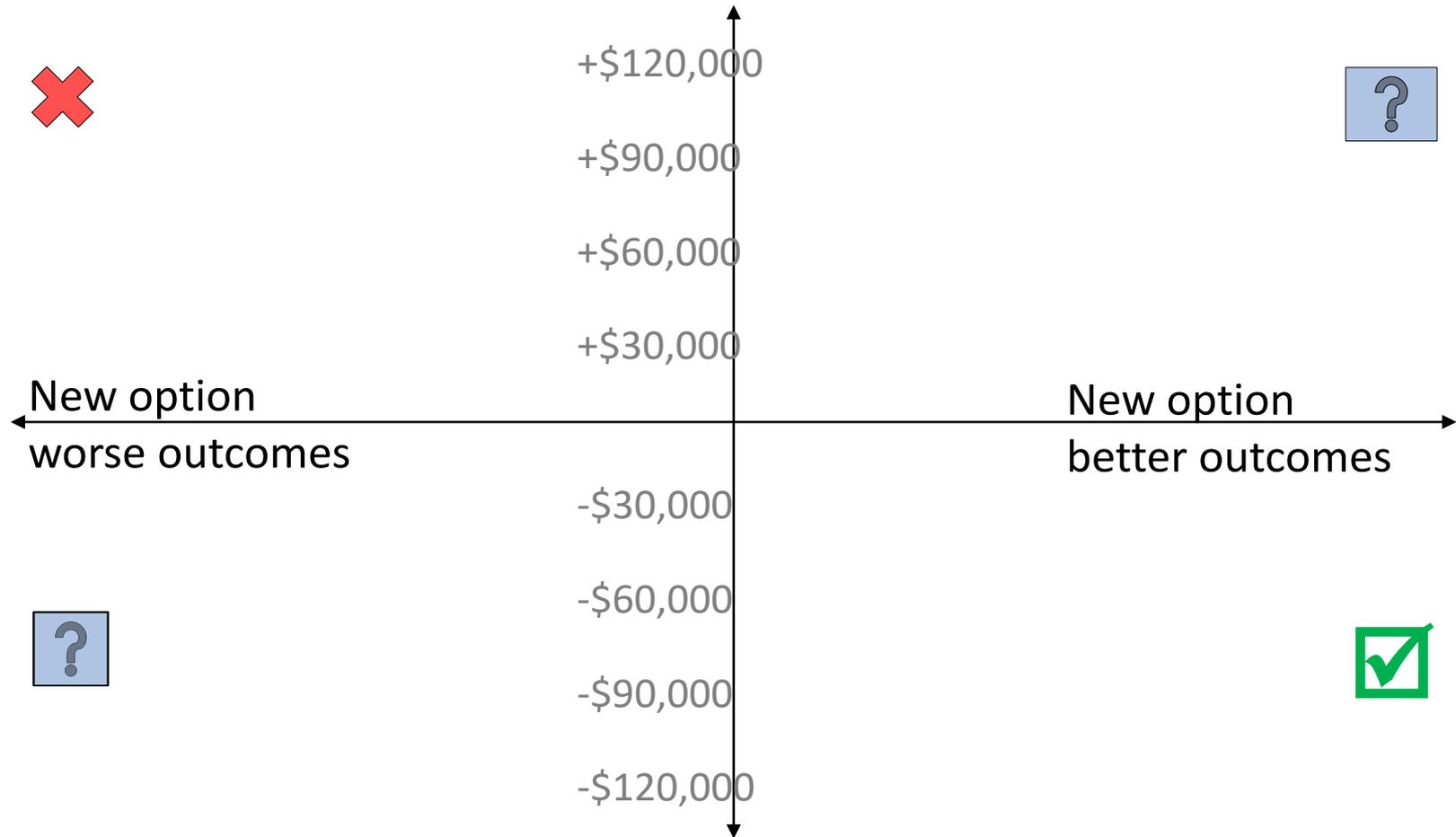
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Economic evaluation

Cost-consequence analysis

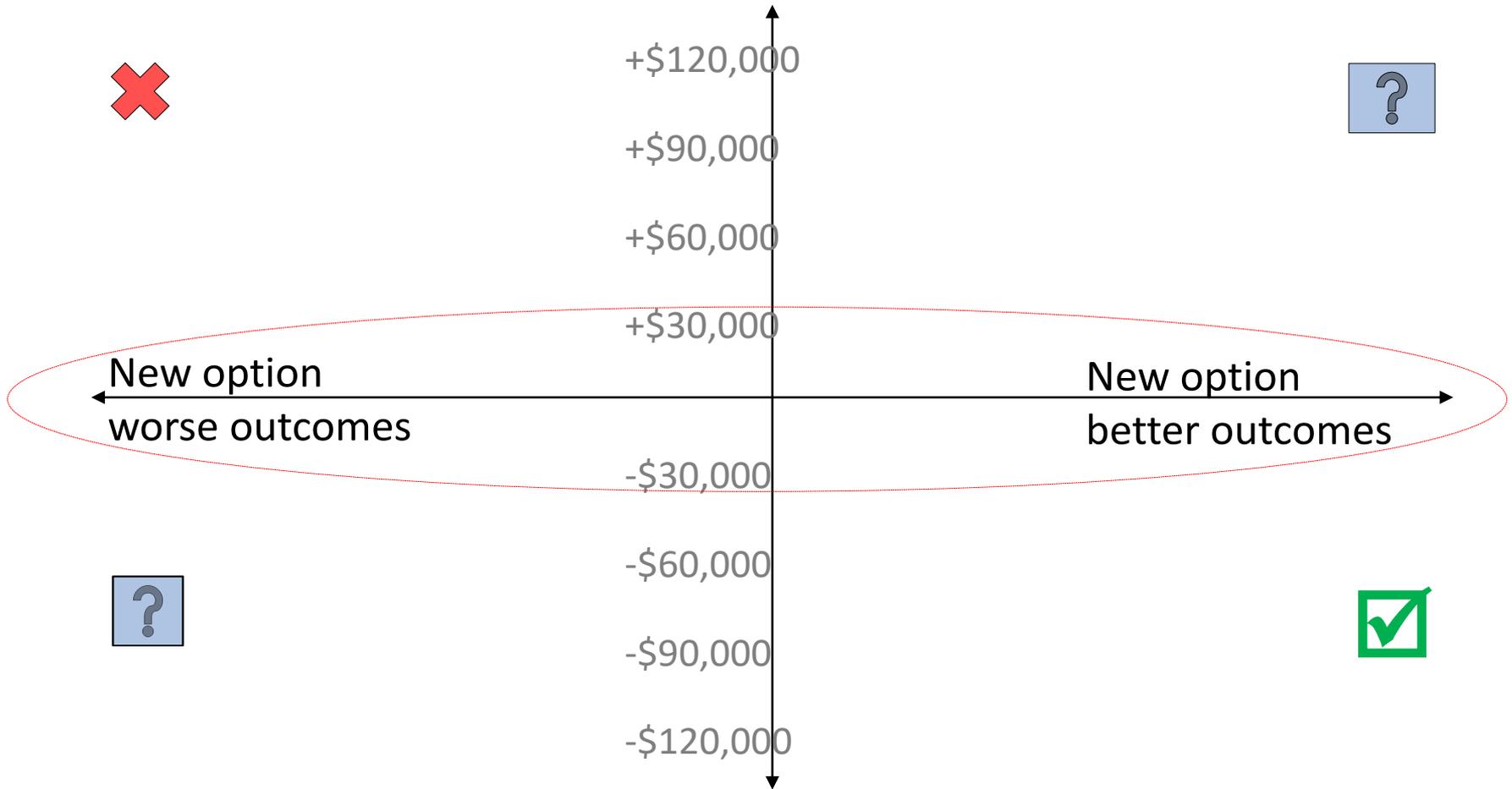# Economic evaluation

Cost-consequence analysis

Economic evaluation

Cost-consequence analysis

Idea of the QALY

What should we fund?

- How is the ___consequence___ part of cost-consequence analysis measured?

  - Easy to specify a bilateral comparison of the two treatments have the same goal, e.g. ibuprofen and paracetamol

  - But how do you compare the effectiveness of, say, hip replacement surgeries versus child vaccinations?

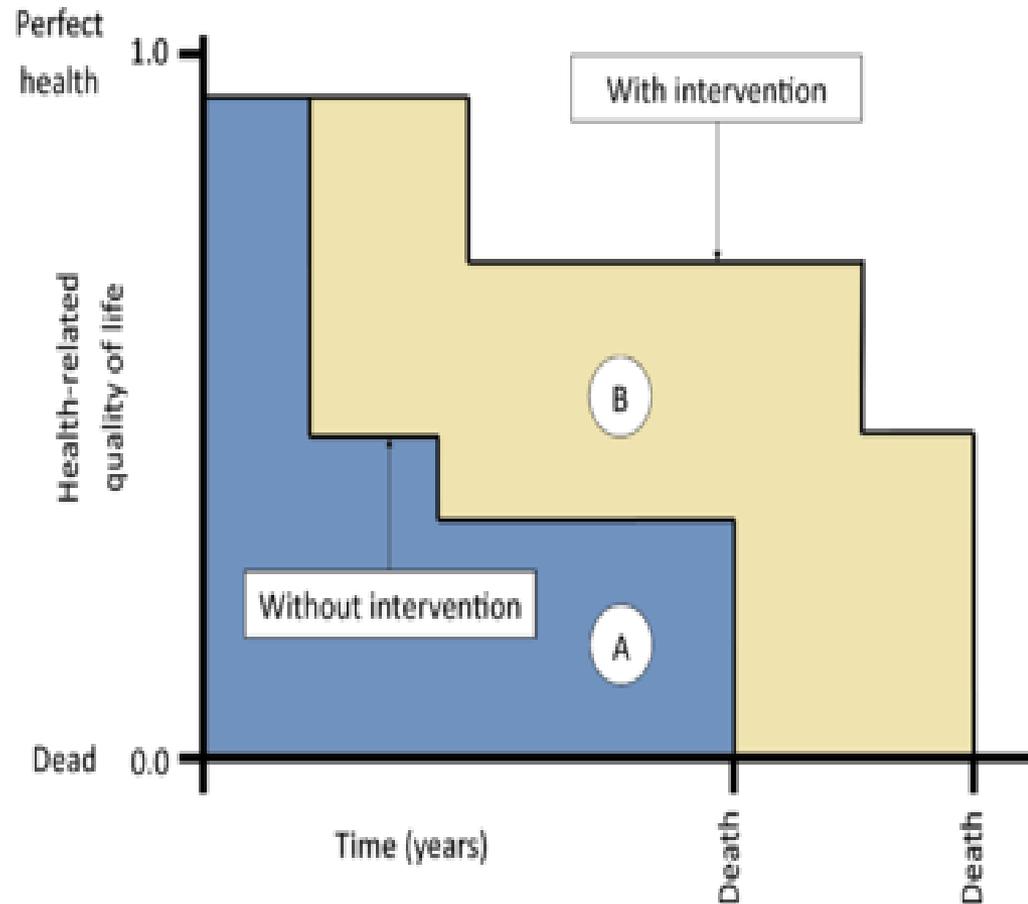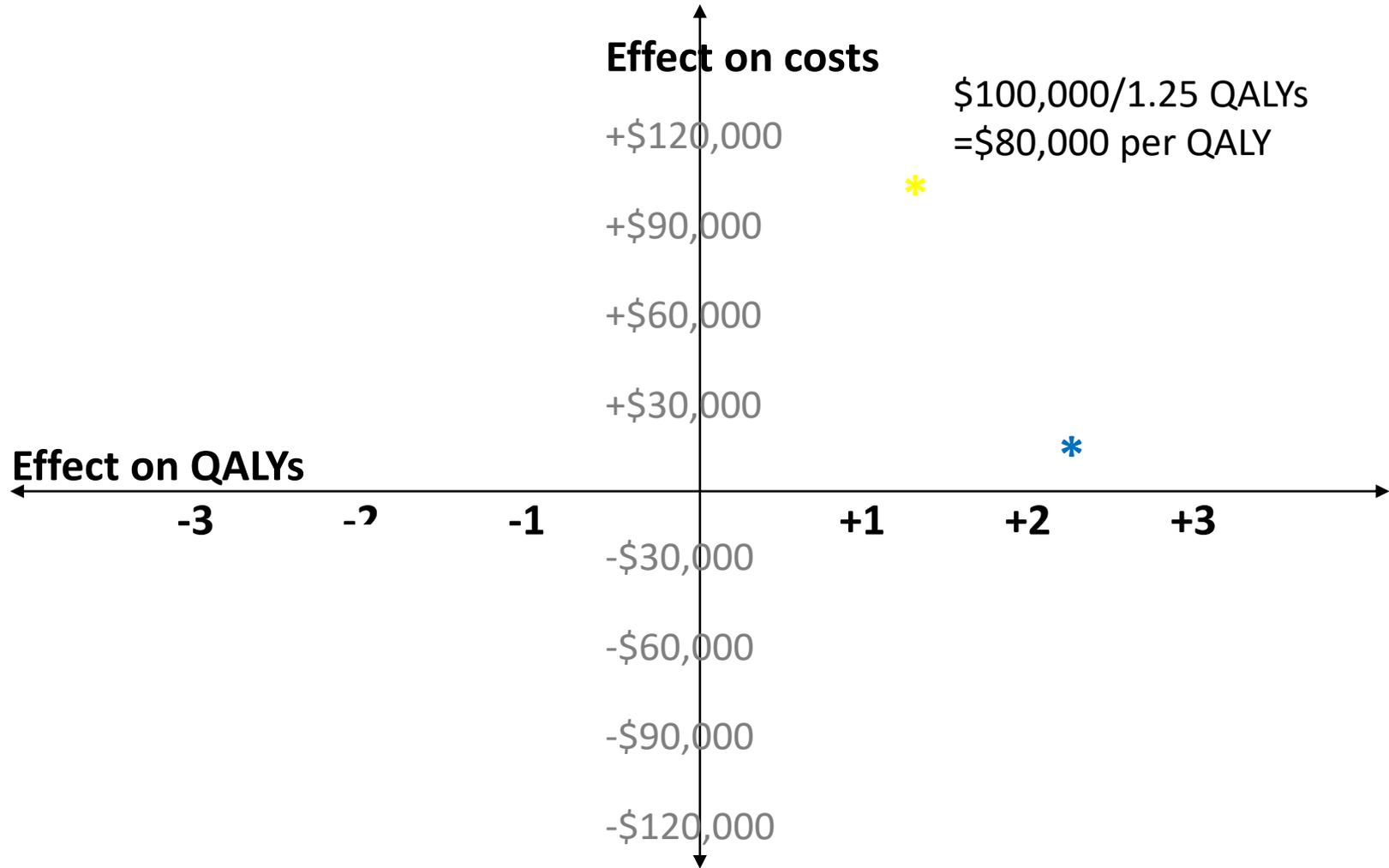 - Allocating a system-wide budget requires a vast number of such comparisons

Idea of the QALY

Quality-**A**djusted **L**ife **Y**ear:
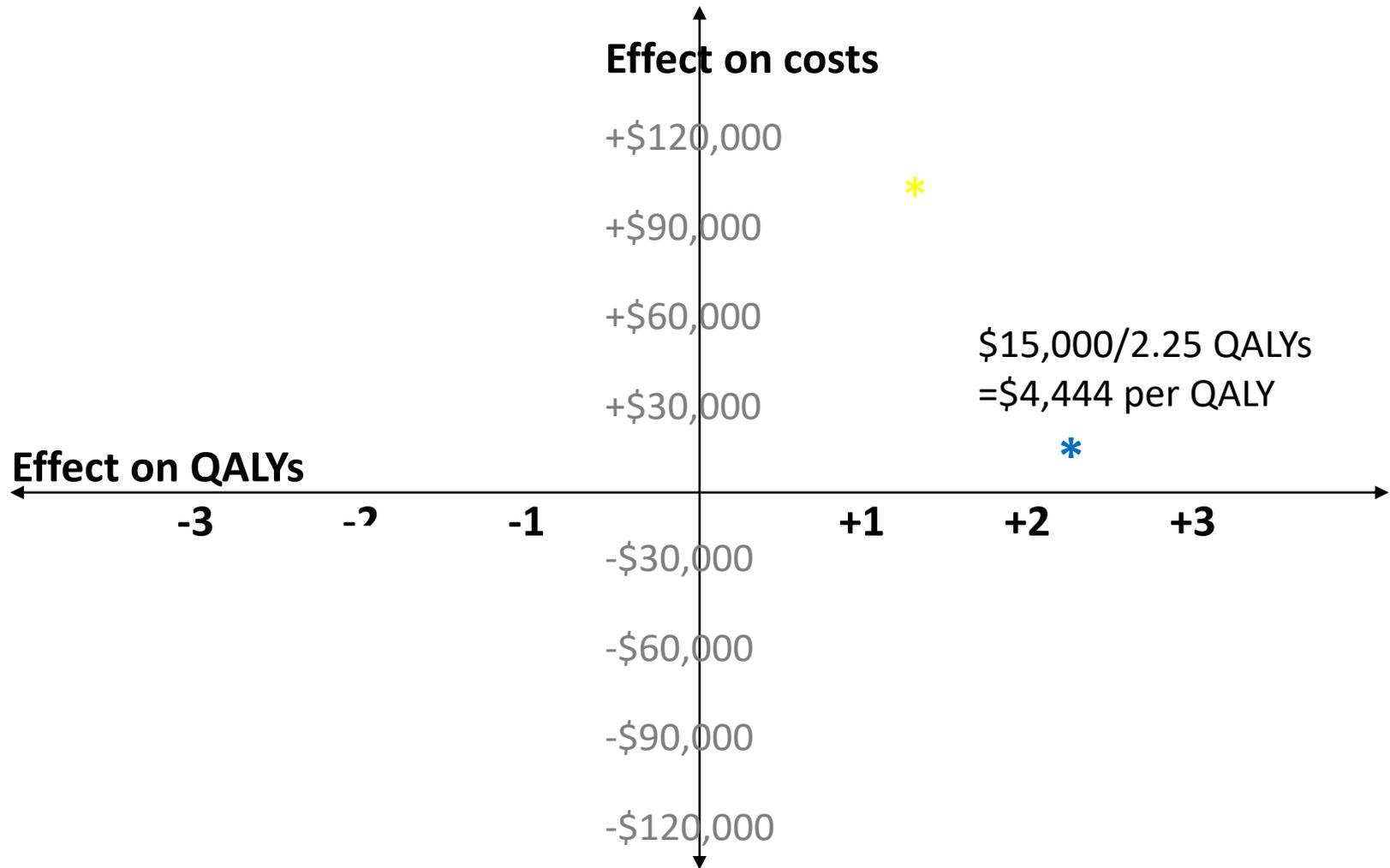
A **generic** measure combining HRQoL and survival, where:

- Health can be indexed on y axis, time on the x

- y*x gives QALY total

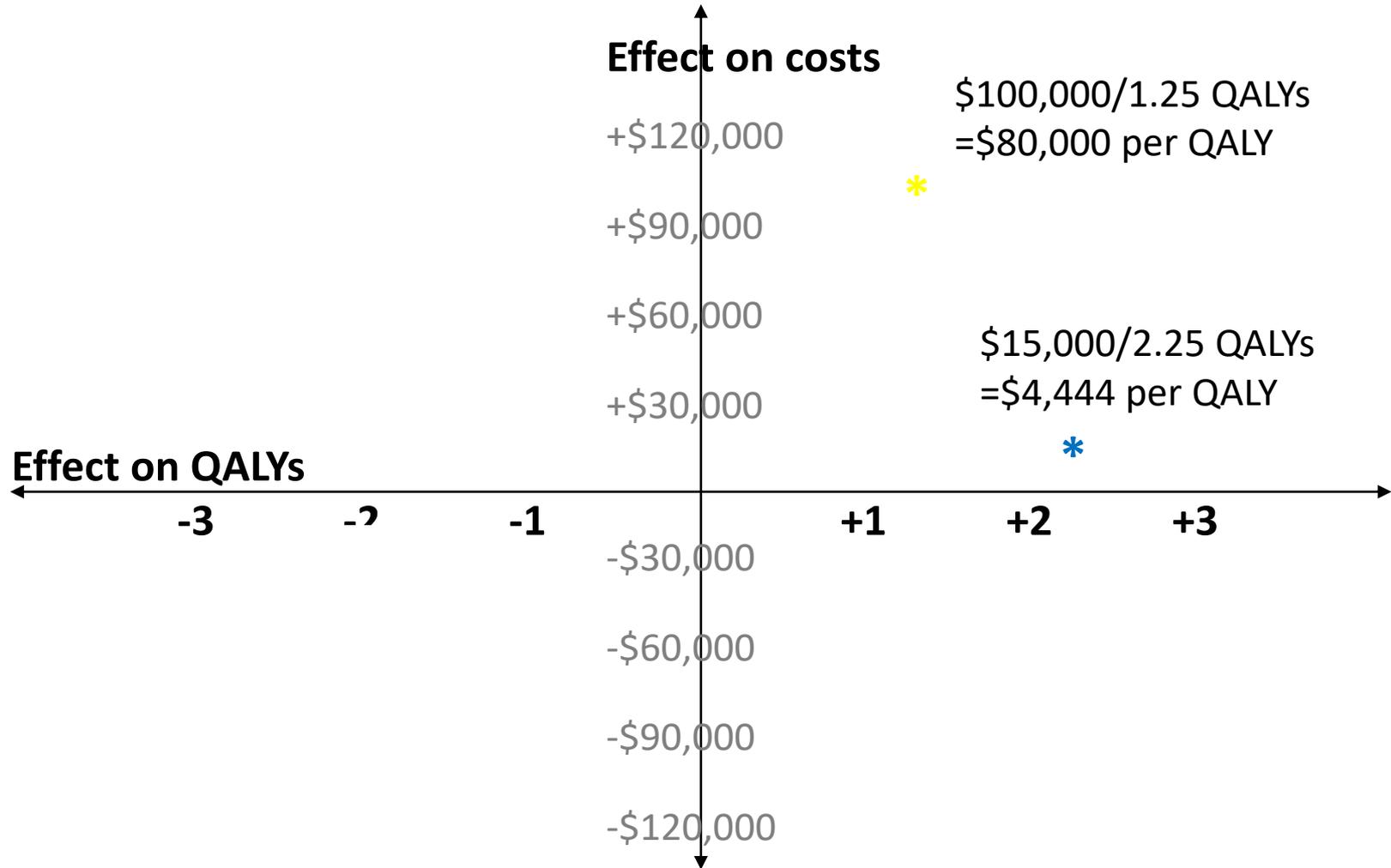- One QALY is equivalent to 12 months in perfect health (or 24 months at 50% of perfect health, etc)

Idea of the QALY

Effect on costs

$100,000/1.25 QALYs
=$80,000 per QALY

+$120,000

+$90,000

+$60,000

+$30,000

Effect on QALYs

-3   -2   -1   +1   +2   +3

-$30,000

-$60,000

-$90,000

-$120,000

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Idea of the QALY

Effect on costs

+$120,000

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+$60,000

$15,000/2.25 QALYs
=$4,444 per QALY

+$30,000

Effect on QALYs

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Idea of the QALY

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Effect on QALYs

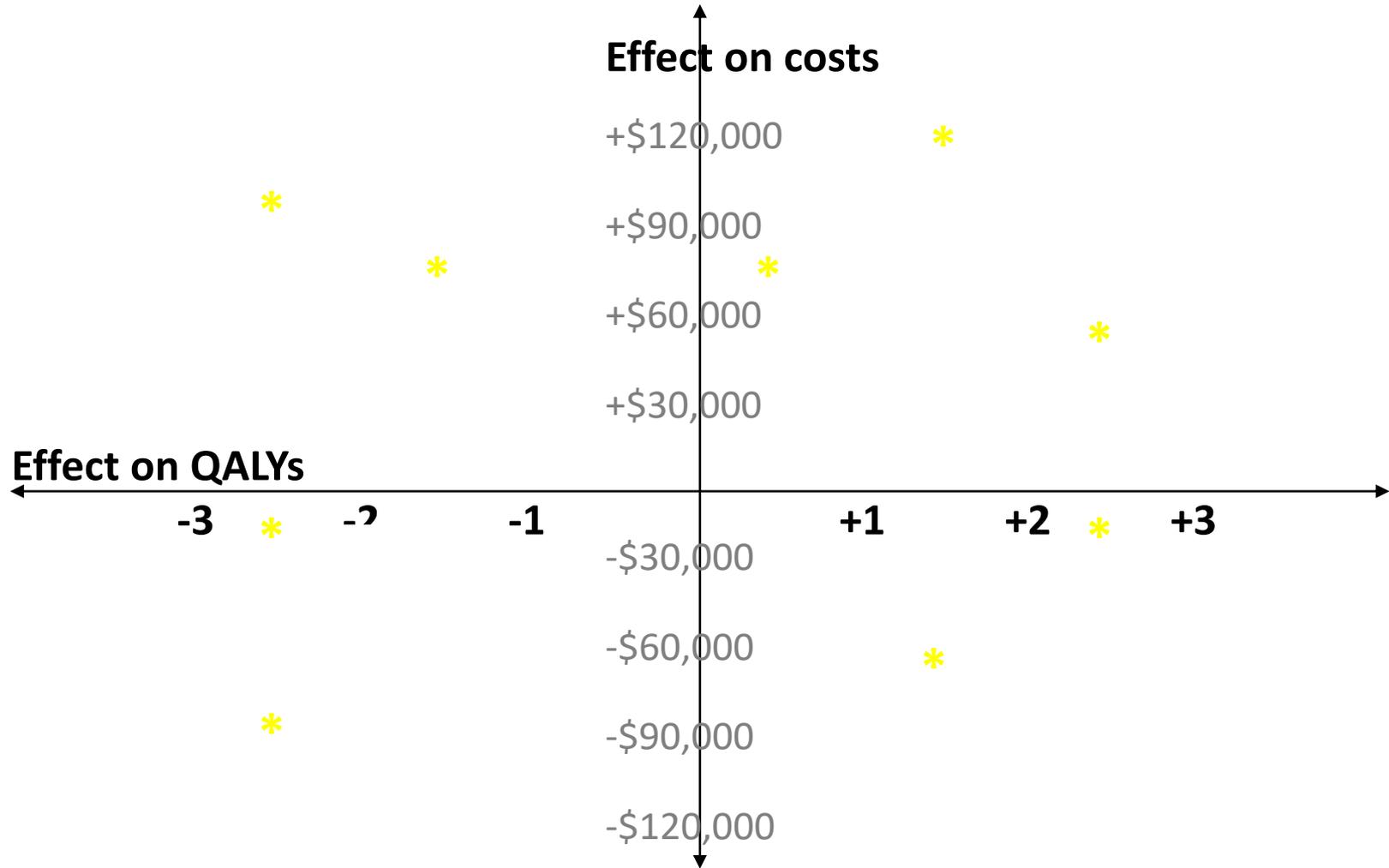-3   -2   -1   +1   +2   +3

-$30,000

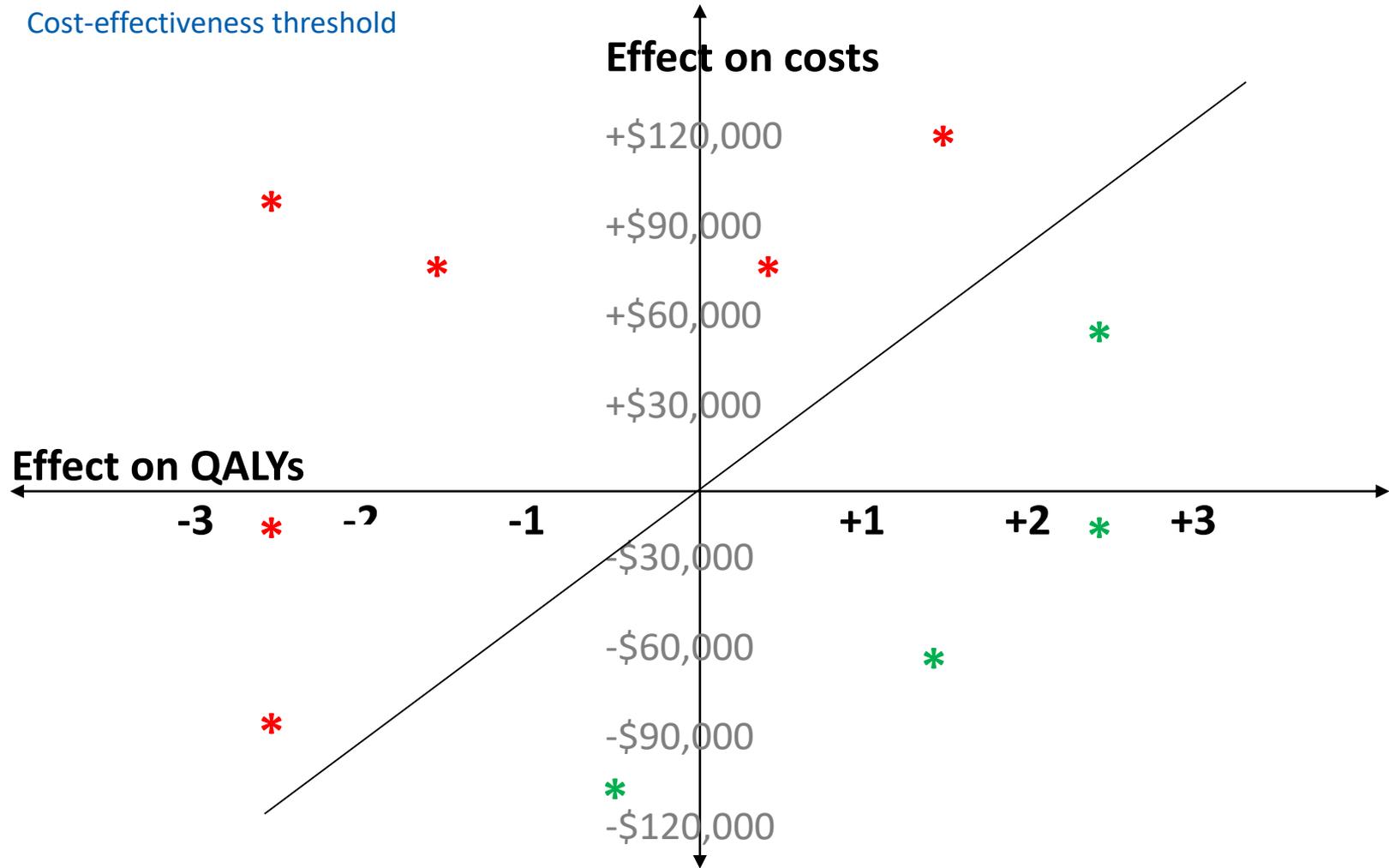-$60,000

-$90,000

-$120,000

Idea of the QALY

Idea of the QALY

Cost-effectiveness threshold

The 'QALY problem' in Palliative Care
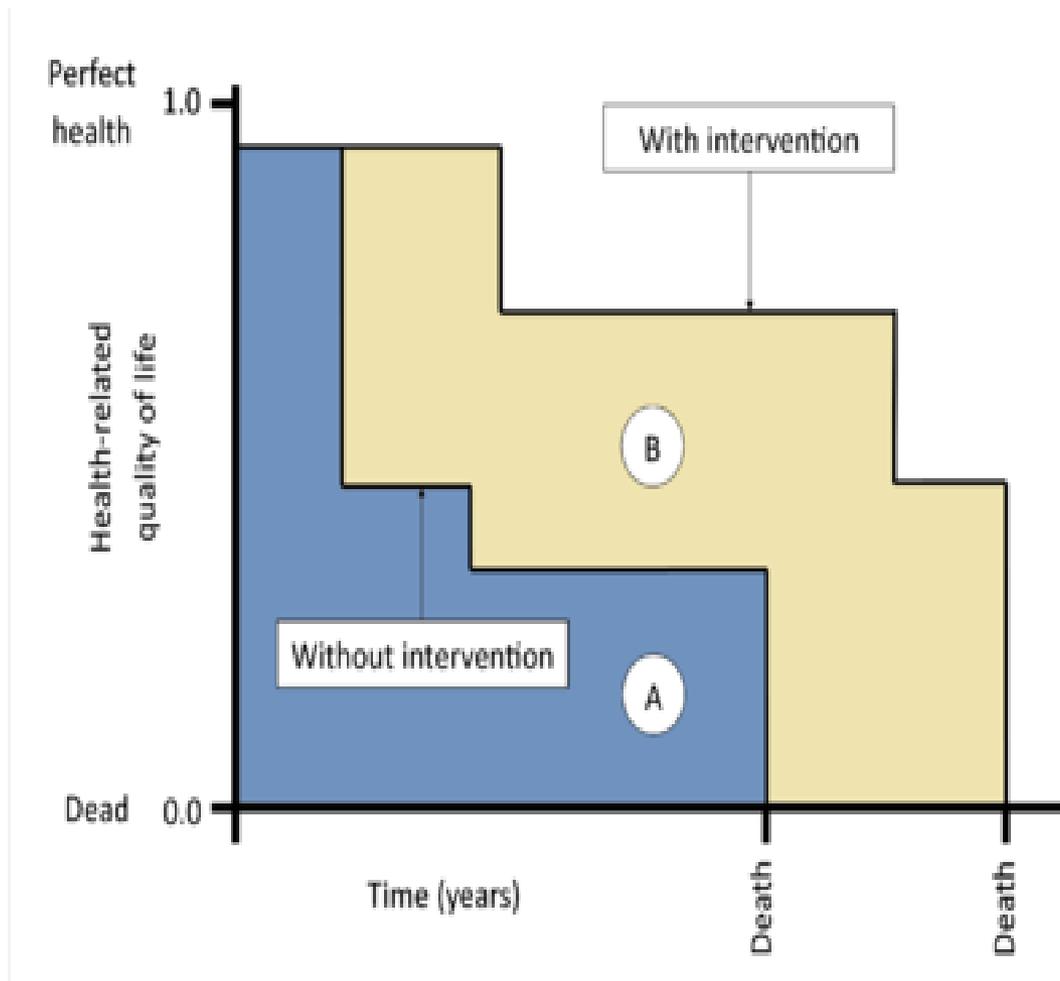
Problems in the EOL context

QALY approach has controversies, e.g. equity

In addition, there are concerns specific to EOL context.

- General bias: PC may not impact survival, have relatively short-term impact on QoL

- Measurement issues:

    - QALYs assume additive time

The 'QALY problem' in Palliative Care

The 'QALY problem' in Palliative Care

Problems in the EOL context

In addition to general limitations to QALY analysis, there are concerns specific to EOL context.

- General bias: PC may not impact survival, have relatively short-term impact on QoL

- Measurement issues:

  - QALYs assume additive time, **but some evidence EOL time is valued differently**
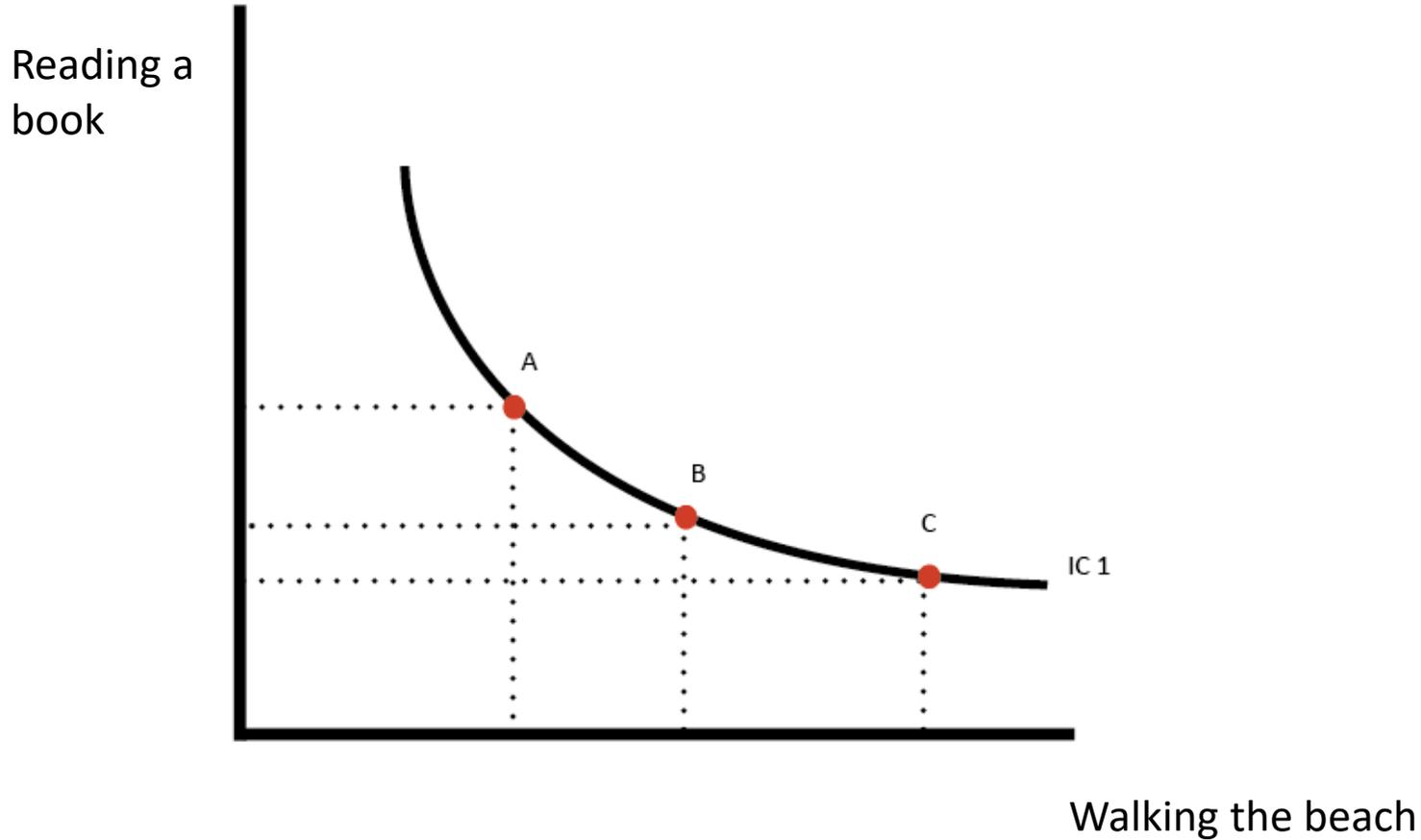
The 'QALY problem' in Palliative Care

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    - QALYs assume trade-able preferences

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Preferences

Indifference curves

The 'QALY problem' in Palliative Care

Problems in the EOL context

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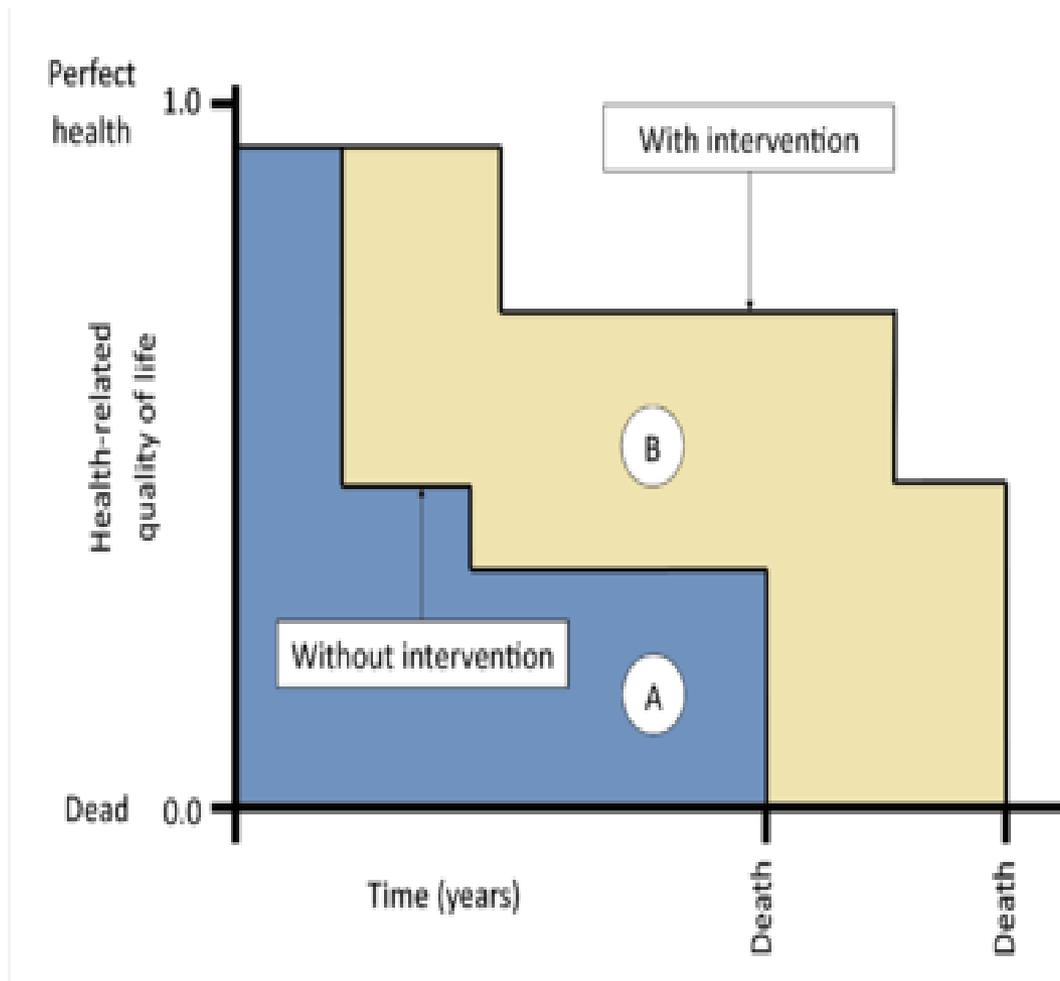
The 'QALY problem' in Palliative Care

In addition to general limitations to QALY analysis, there are concerns specific to EOL context.

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- Measurement issues:

  - QALYs assume additive time, but some evidence EOL time is valued differently

  - QALYs assume trade-able preferences, but some evidence EOL preferences are lexicographical

  - QALYs can't cope with "states worse than death"

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The 'QALY problem' in Palliative Care

The 'QALY problem' in Palliative Care

Some reading

- There is a small, lively literature on this for those who are interested.

- A good starting point/general overview:

  WICHMANN, A et al. 2017. The use of Quality-Adjusted Life Years in cost-effectiveness in palliative care. Pal Med, 31(4), 306-322.

- A hard-nosed economist's defence of the QALY and lots of references to other viewpoints, is:

  ROUND, J. 2012. Is a QALY still a QALY at the end of life? J Health Econ, 31, 521-7.

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Economic evaluation

A note on US *realpolitik*

- Different systems use EE in different ways

- NHS perhaps the most explicit, via NICE (nice.org.uk)

- In the US, formal use is limited and confusing

  - Some funding bodies forbid EE ('bureaucratic rationing')

  - Heightened sensitivity @EOL ("death panels")

  - PC in US has not grown in a rational, planned way

Economic evaluation

A note on US *realpolitik*

- However, the intellectual ground is solid:

  - Rationing inevitable in all systems due to <u>scarcity</u>

  - EE therefore essential to ethical health policy

  - Most opposition reflects broader bad faith vs. UHC

  - Foundational textbooks in the US and UK are v. v. similar

  - ➢ US h/care dysfunction may limit impact of highest-quality economic evaluations but do not lose sight of fundamental principles

Economic evaluation in EOL care

Summary

- Cost-consequence analysis is a key gap in current EOL literature

- Mainly reflects practical & methodological issues

- Long-term development of evidence, services demands CCA

- Political controversies do not diminish intellectual and ethical imperatives

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End of part one

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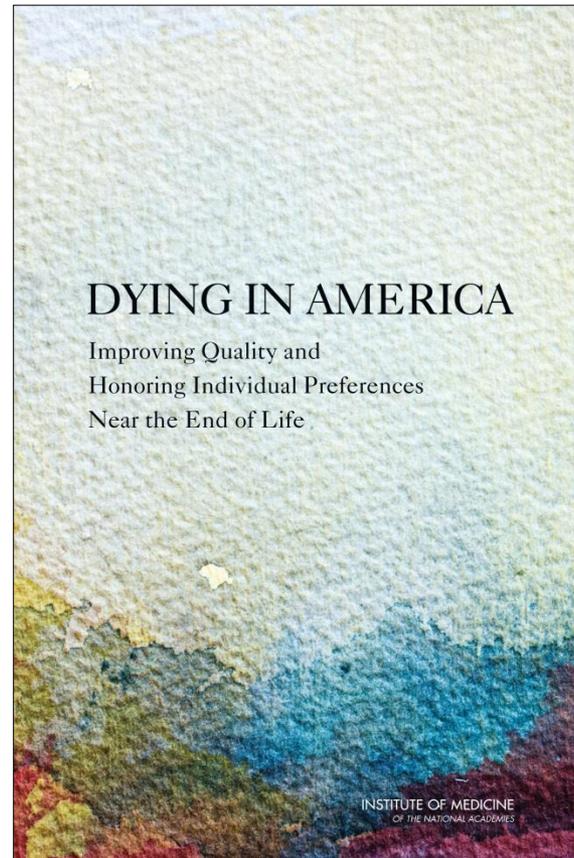## Part 2: Key issues in the evidence base (Aldridge)

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Dying in America: Improving Quality and Honoring Individual Preferences Near the End of Life

IOM (Institute of Medicine). 2014. *Dying in America: Improving quality and honoring individual preferences near the end of life.* Washington, DC: The National Academies Press.

Commissioned paper: the "Ask"

Provide an analysis of the epidemiology of serious illness and high utilization of healthcare
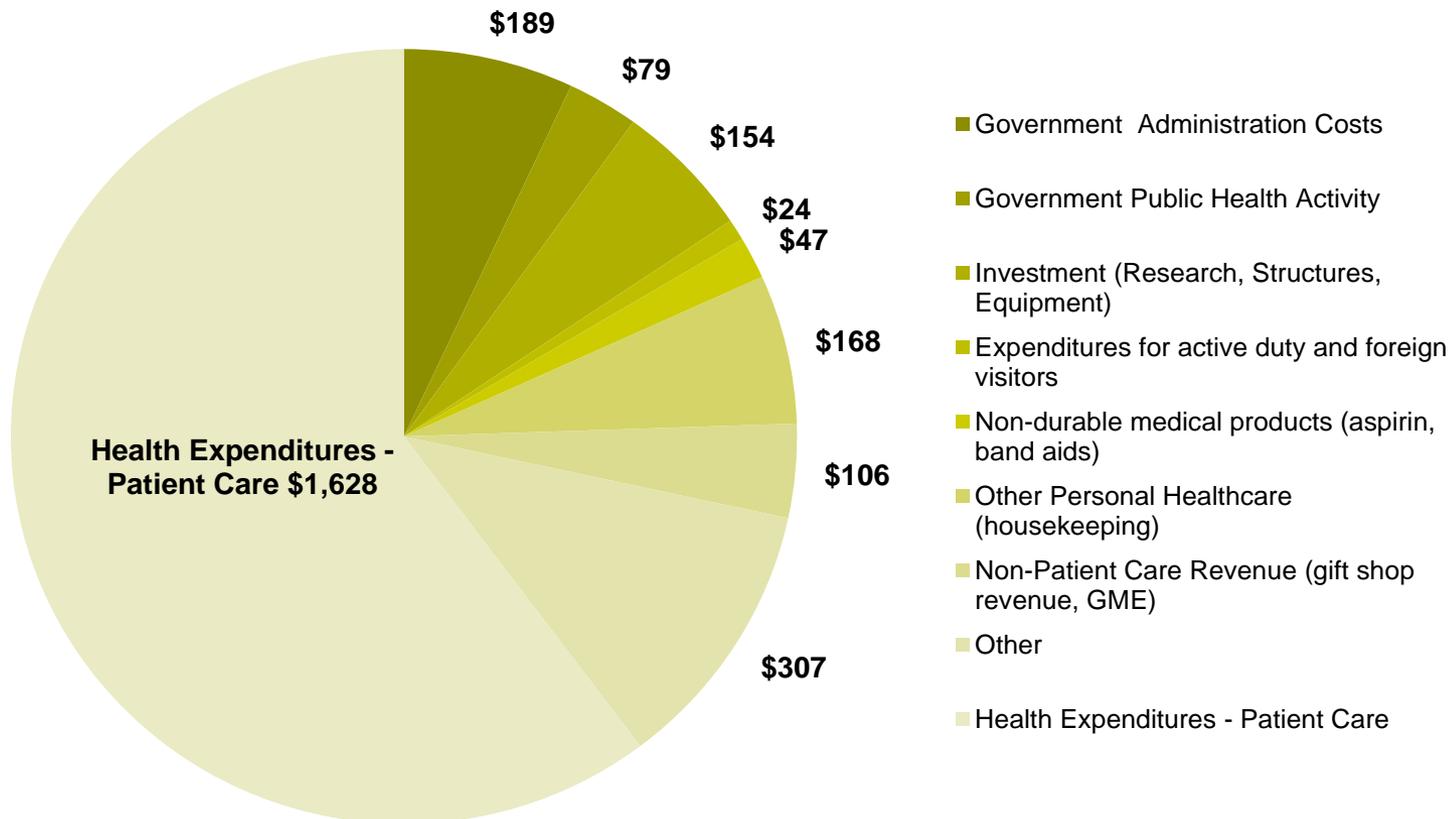
Synthesize and augment existing evidence to

➢ Evaluate costs and intensity of healthcare for individuals who have died

➢ Characterize the population that utilizes the most healthcare ("high cost" group)

➢ Provide an analysis of the overlap between these two groups

Identify gaps in what is known and how results of the analysis will inform policy

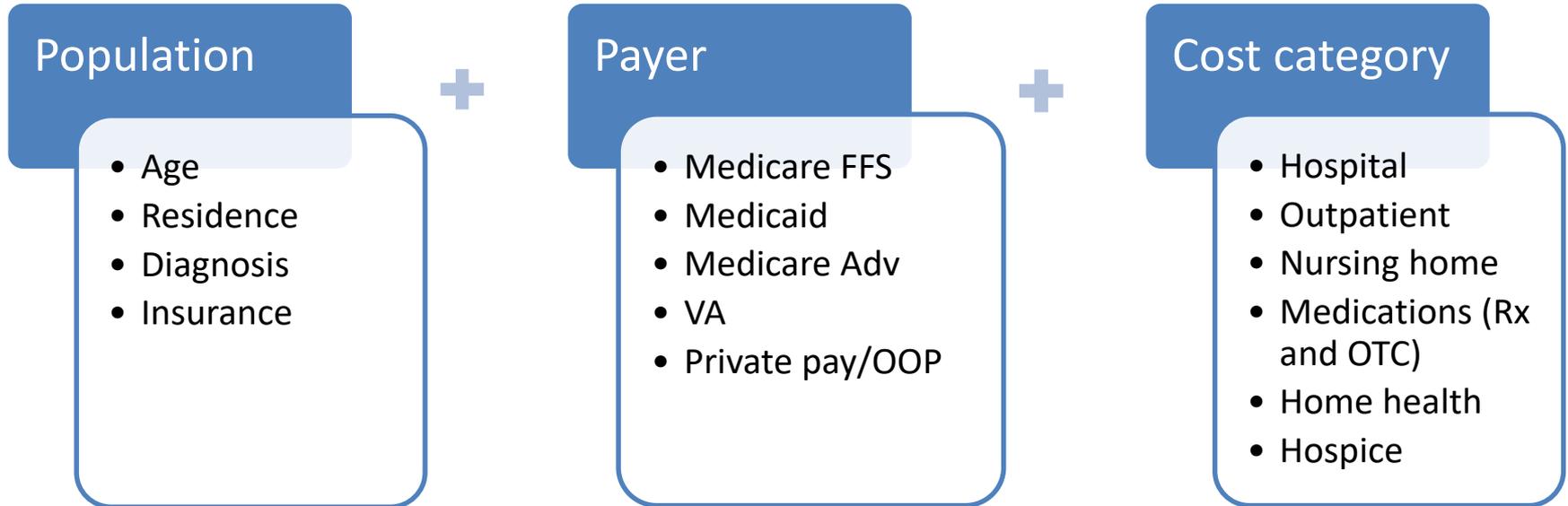# Healthcare reform debate in the context of healthcare costs

1. Discussion of high __total__ healthcare costs and reform proposals on how to decrease total costs

2. Discussion of __growth__ in healthcare costs and reform proposals aimed at "bending" the costs curve

3. Discussion of the highly __concentrated__ healthcare costs among a small proportion of the population and policy proposals to identify and target this "high cost" group

Components of the $2.7 Trillion of National Health Expenditures, 2011

$189
$79
$154
$24
$47
$168
$106
$307

Health Expenditures - Patient Care $1,628

- Government Administration Costs
- Government Public Health Activity
- Investment (Research, Structures, Equipment)
- Expenditures for active duty and foreign visitors
- Non-durable medical products (aspirin, band aids)
- Other Personal Healthcare (housekeeping)
- Non-Patient Care Revenue (gift shop revenue, GME)
- Other
- Health Expenditures - Patient Care

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Healthcare cost data?

Population
- Age
- Residence
- Diagnosis
- Insurance

+

Payer
- Medicare FFS
- Medicaid
- Medicare Adv
- VA
- Private pay/OOP

+

Cost category
- Hospital
- Outpatient
- Nursing home
- Medications (Rx and OTC)
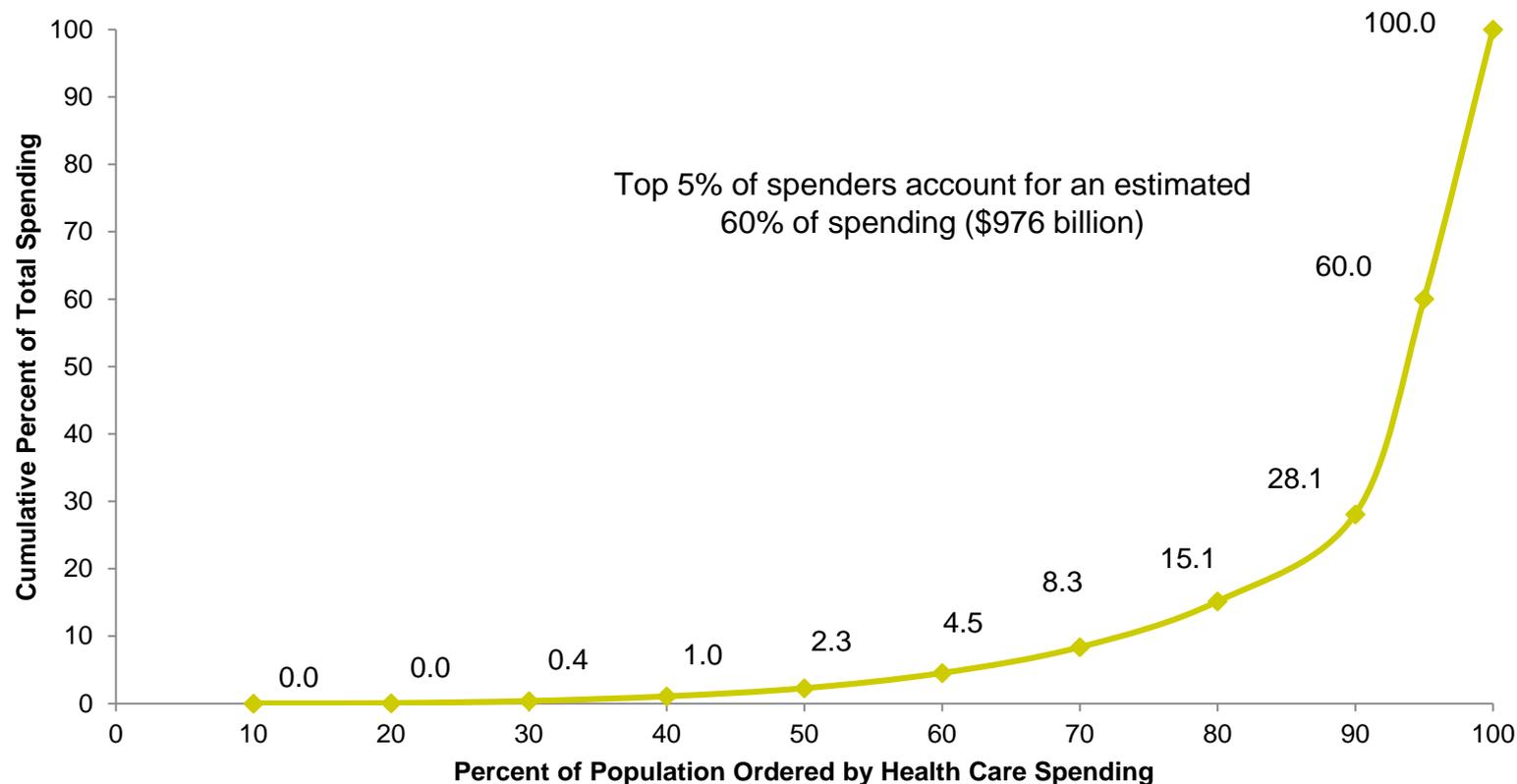- Home health
- Hospice

Total annual healthcare expenditures

Medical Expenditures Panel Survey (MEPS)

– set of large-scale surveys of families and individuals, their medical providers, and employers across the United States. MEPS is the most complete source of data on the cost and use of health care and health insurance coverage

Annual healthcare expenditures of the non-community dwelling U.S. population, primarily the nursing home population, imputed from National Health Expenditure Data, National Center for Health Statistics data, and peer-reviewed literature
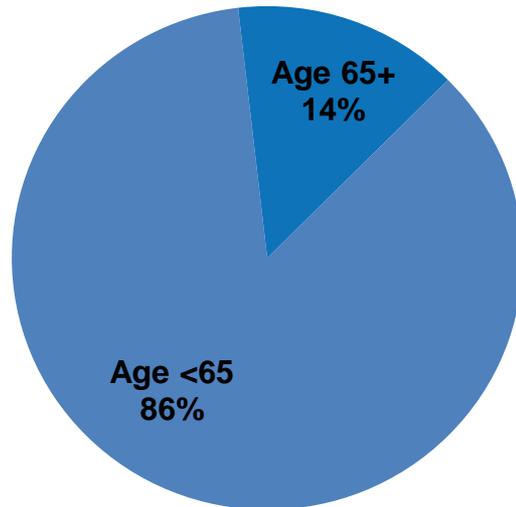
Cumulative Distribution of Personal Health Care Spending ($1.6 trillion), 2011

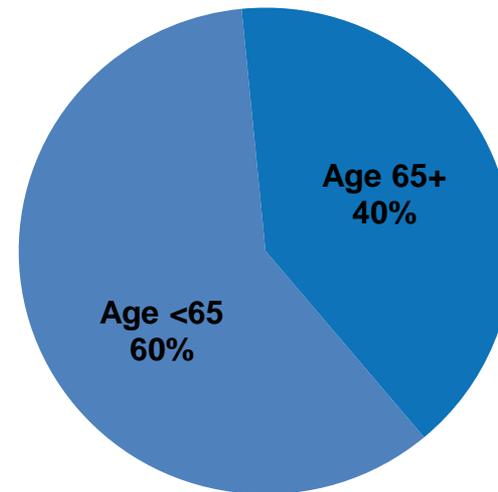Top 5% of spenders account for an estimated 60% of spending ($976 billion)

- Source: Aldridge, Kelley, 2013: IOM Commissioned Paper: Epidemiology of Serious Illness and High Utilization of Healthcare
- Note: Total population and healthcare costs obtained from 2011 Medical Expenditure Panel Survey data adjusted to include the nursing home population. The entire nursing home population is estimated to be in the top 5% of total healthcare spending.
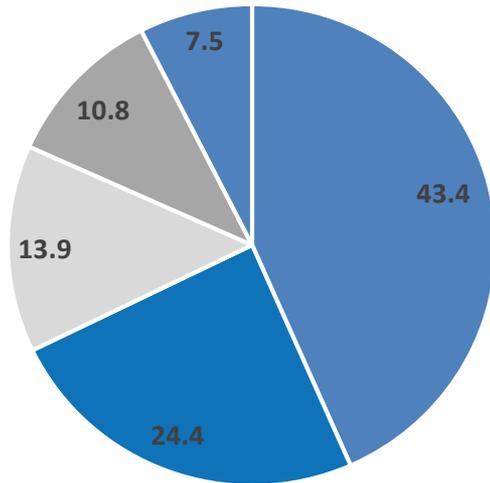
Age and Healthcare Costs

Total Population, By Age

Age 65+
14%

Age <65
86%

High-Cost Population, By Age
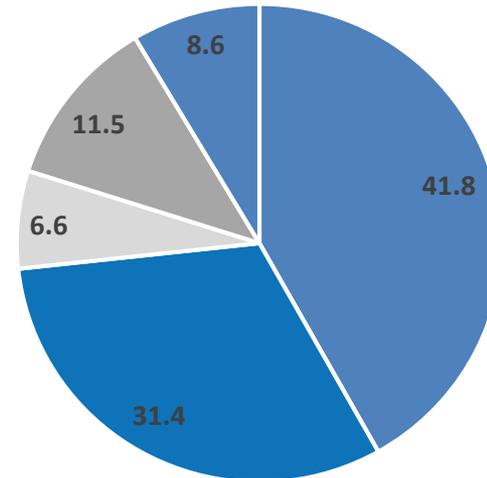

Age 65+
40%

Age <65
60%

- Although individuals aged 65+ are disproportionately in the top 5% of healthcare spenders, almost 2/3rds of the top 5% spenders are younger than 65
- Older age is a *risk factor* for higher healthcare costs, but older adults make up the minority of high cost spenders

Payor and Healthcare Costs

Total Healthcare Costs, 2011

Healthcare Costs for Top 5%, 2011

■ Private ■ Medicare ■ Out of Pocket ■ Medicaid ■ Other

- Similar proportions of healthcare costs in total and for the high cost group for private insurance and Medicaid
- Higher proportion of healthcare costs for the high cost group is paid by Medicare and a lower proportion OOP

Population and Healthcare Costs by Existence of Chronic Conditions and Functional Limitations

Total Population	No. People (mil)		Healthcare costs (bil)	
No chronic conditions or functional limitations	149.3	48%	$186.3	11%
Chronic conditions only	112.0	36%	$505.7	31%
Functional limitations only	6.2	2%	$26.6	2%
Chronic conditions and functional limitations	44.9	14%	$908.8	56%

Although the presence of chronic conditions is a key driver of healthcare costs, the addition of functional limitations appears to differentiate a high-cost group within those with chronic conditions
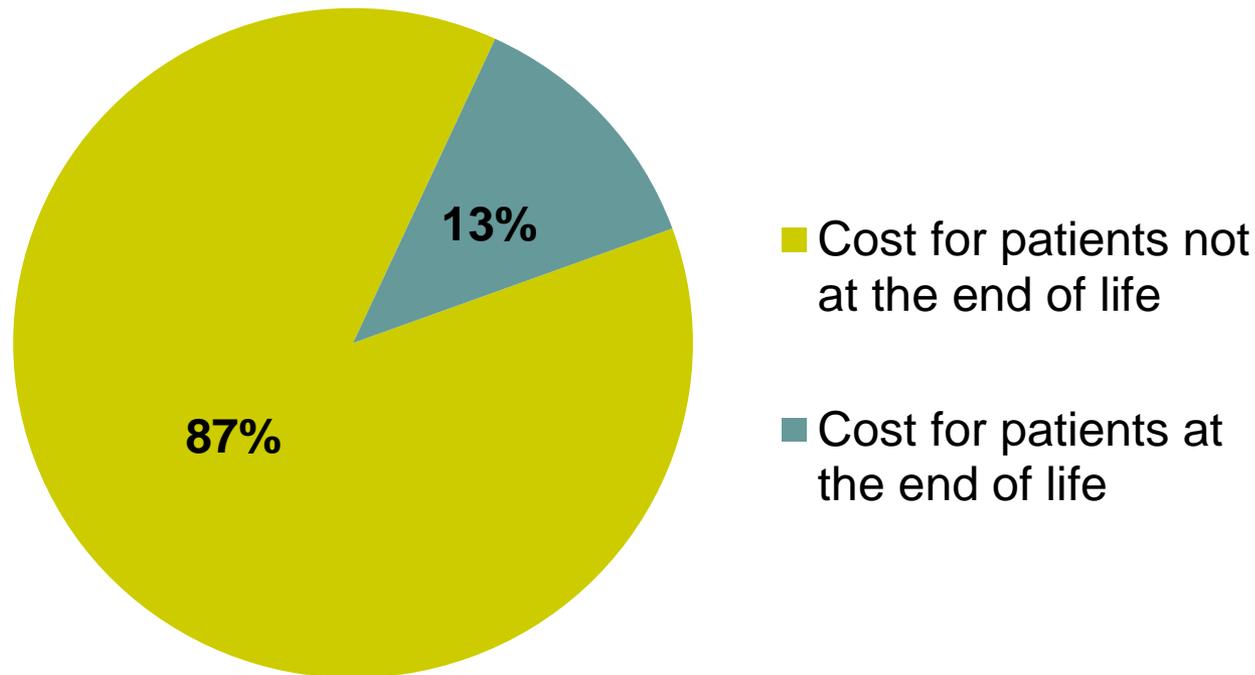
Cost of Care at the End of Life

How much are total healthcare costs for people in their last year of life?

Of the population in the "high cost" group [those we potentially want to target for intervention] how many are in their last year of life? [overlap question]

Proportion of Total Healthcare Costs for Patients at the End of Life
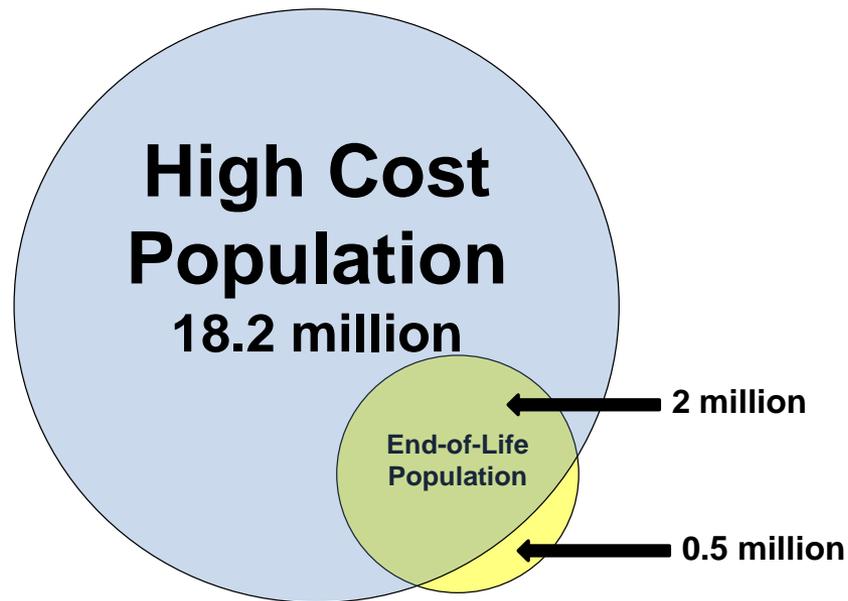

- Cost for patients not at the end of life
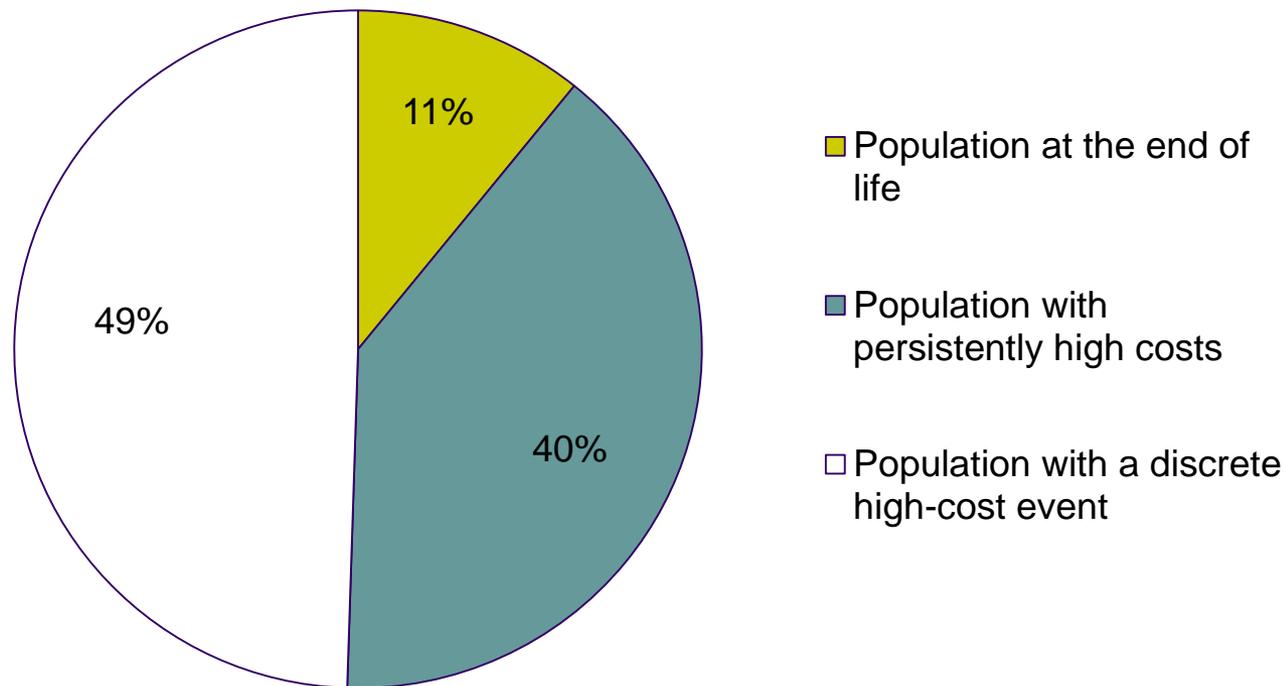- Cost for patients at the end of life

87%

13%

- Source: Aldridge, Kelley, 2013: IOM Commissioned Paper: Epidemiology of Serious Illness and High Utilization of Healthcare
- Note: The total pie represents total personal healthcare costs of $1.6 trillion

Estimated Overlap Between the Population with the Highest Healthcare Costs and the Population at the End of Life

High Cost Population
18.2 million

End-of-Life Population

2 million

0.5 million

• Source: Aldridge, Kelley, 2013: IOM Commissioned Paper: Epidemiology of Serious Illness and High Utilization of Healthcare

Population with the Highest Healthcare Costs (Top 5%) by Illness Trajectory

- Population at the end of life
- Population with persistently high costs
- Population with a discrete high-cost event

11% | 40% | 49%

• Source: Aldridge, Kelley, 2013: IOM Commissioned Paper: Epidemiology of Serious Illness and High Utilization of Healthcare

Projected Cost Savings of Hypothetical Interventions By Target Population

Target Population	Population Size	Total Costs ($bil)	Intervention	% of Population Impacted by Intervention	Potential Reduction in Healthcare Costs (%)	Potential Reduction in Healthcare Costs ($bil)
Age >=65 with chronic conditions and functional limitations	22,092,740	$543	A	50%	10%	$27
			B	50%	5%	$14
All individuals with chronic conditions and functional limitations	44,946,847	$909	A	50%	10%	$45
			B	50%	5%	$23
Individuals at the end of life	2,468,435	$200	A	50%	10%	$10
			B	50%	5%	$5

• Source: Aldridge, Kelley, 2013: IOM Commissioned Paper: Epidemiology of Serious Illness and High Utilization of Healthcare

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End of part two

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Current evidence

Cost of care for serious illness

- 2001-2011: US healthcare spending doubled

- By 2040, projected to be 1/3 of all economic activity in the US

- Similar, less dramatic trends in other HICs and LMICs

- LYOL is most expensive **<u>BUT</u>** high costs driven those with long-term chronic conditions and functional limitations (Aldridge & Kelley, 2015, Davis et al., 2016)

  ➢ Lowering costs for those with serious and complex medical illness is key to US health system sustainability

Current evidence

Four key systematic literature reviews

Review	Key findings
Smith et al. (2014)	• All settings, study designs; 46 papers • General pattern of cost-saving, heterogeneity of everything
Langton et al. (2014)	• Count-back studies of administrative data; 78 (!) papers • Lower costs for PC, increasing use of 'decedent cohort' design
Gomes et al. (2013)	• High quality studies of homecare; 6 economics papers • ~15-30% cost-saving
May et al. (2014)	• Prospective studies of hospital inpatient PCC; 10 papers • ~15-20% cost-saving (see also May 2018 meta-analysis)

Current evidence

- Together these reviews establish two points of consensus:
    1. Palliative care is associated with lower health care/system costs
    2. Knowledge gaps re:
        - Everything! Few meta-analyses (so far)
        - But in particular **limited scope of enquiry**:
            i. Analytic framework and the QALY problem
            ii. Timeframe
            iii. Perspective
            iv. Intervention timing (and what is "palliative care" anyway?)

Current evidence

Limitation (ii): Timeframe

- Most evidence is from one of two phases of care:

  - Inpatient hospital stays

  - End of life (decedent count-back studies)

- Both associated with intensive treatment

  - Not representative of full trajectory of serious illness

  - Observational designs (so concerns re: matching)

  - EOL data a concern (Bach et al., 2004; Earle & Ayanian, 2006)

Current evidence

Temel (2010): RCT of palliative care from diagnosis for NSCLC

Early palliative care
- improves quality of life
- reduces intensity of treatment
- extends survival

ORIGINAL ARTICLE

Early Palliative Care for Patients with Metastatic Non–Small-Cell Lung Cancer

Jennifer S. Temel, M.D., Joseph A. Greer, Ph.D., Alona Muzikansky, M.A., Emily R. Gallagher, R.N., Sonal Admane, M.B., B.S., M.P.H., Vicki A. Jackson, M.D., M.P.H., Constance M. Dahlin, A.P.N., Craig D. Blinderman, M.D., Juliet Jacobsen, M.D., William F. Pirl, M.D., M.P.H., J. Andrew Billings, M.D., and Thomas J. Lynch, M.D.
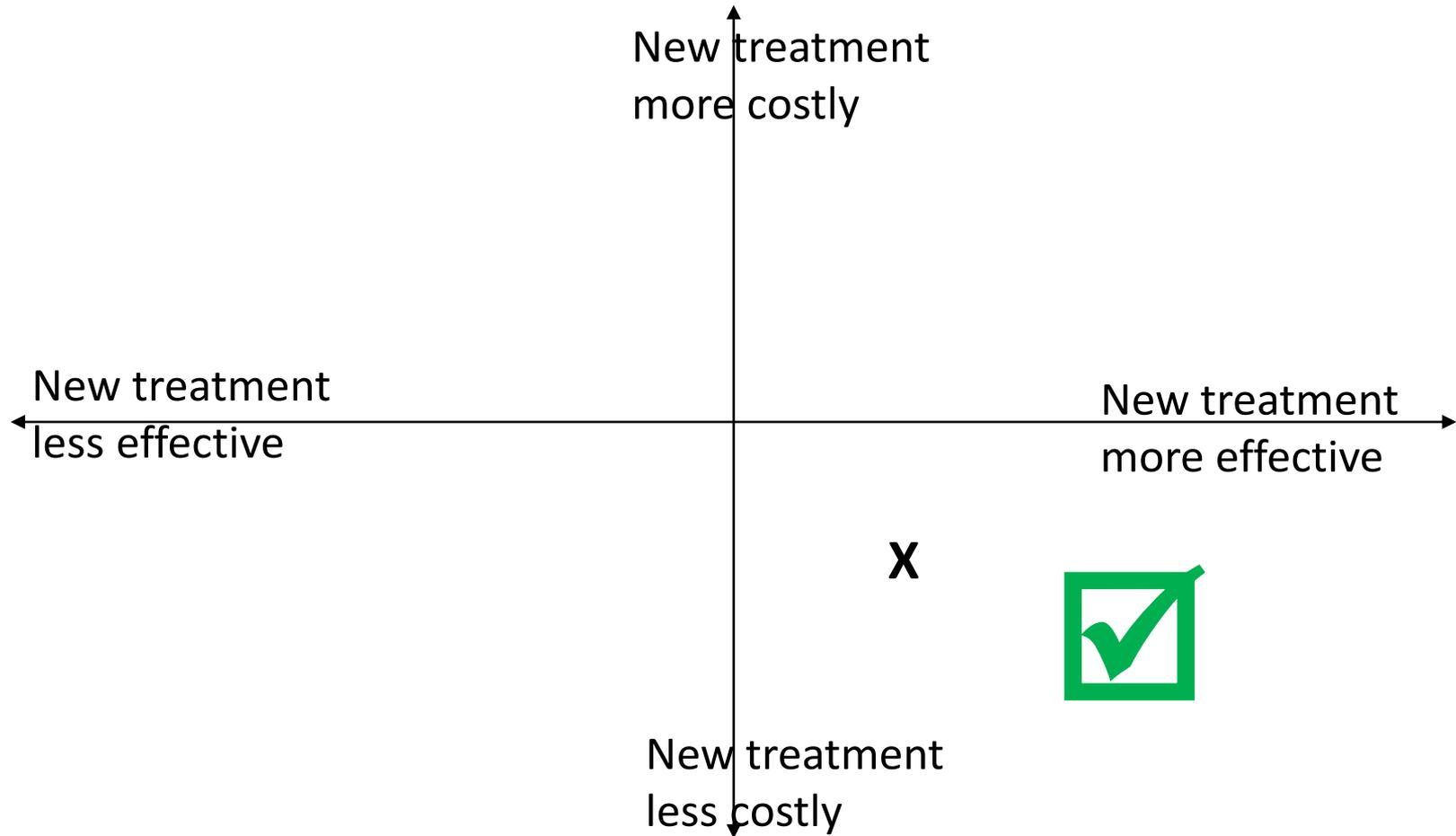
ABSTRACT

BACKGROUND

Patients with metastatic non–small-cell lung cancer have a substantial symptom burden and may receive aggressive care at the end of life. We examined the effect

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Current evidence

Early PC appears a dominant strategy: better outcomes at lower costs

Current evidence

However….

Greer (2016): cost analysis with ~95% of subjects now deceased

Early palliative care
- reduces costs in last 30 days
- increases hospice use
- is associated with higher mean total costs?!

Cost Analysis of a Randomized Trial of Early Palliative Care in Patients with Metastatic Nonsmall-Cell Lung Cancer

Joseph A. Greer, PhD, Angela C. Tramontano, MPH, Pamela M. McMahon, PhD, William F. Pirl, MD, MPH, Vicki A. Jackson, MD, MPH, Areej El-Jawahri, MD, Ravi B. Parikh, MD, Alona Muzikansky, MA, Emily R. Gallagher, RN, and Jennifer S. Temel, MD

Abstract

Background: Several trials have shown that integrated palliative and oncology care improves quality of life and mood in patients with advanced cancers. However, the degree to which early involvement of palliative care (PC) in the outpatient setting impacts the cost of care remains unknown.

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Current evidence

Limitation (ii): Timeframe

Findings such as 'reduced intensity of hospital treatment' and 'lower costs at end of life' are routinely taken in the literature to mean that "palliative care saves money"

So, how is it possible for PC to:

- **reduce initial intensity (weeks 1-12)**

- **reduce cost in the last 30 days of life**

- **increase costs overall?**

Current evidence

Let's look at a simplified data example of two identical patients: one receives UC, one receives PC from point of diagnosis of a terminal disease.
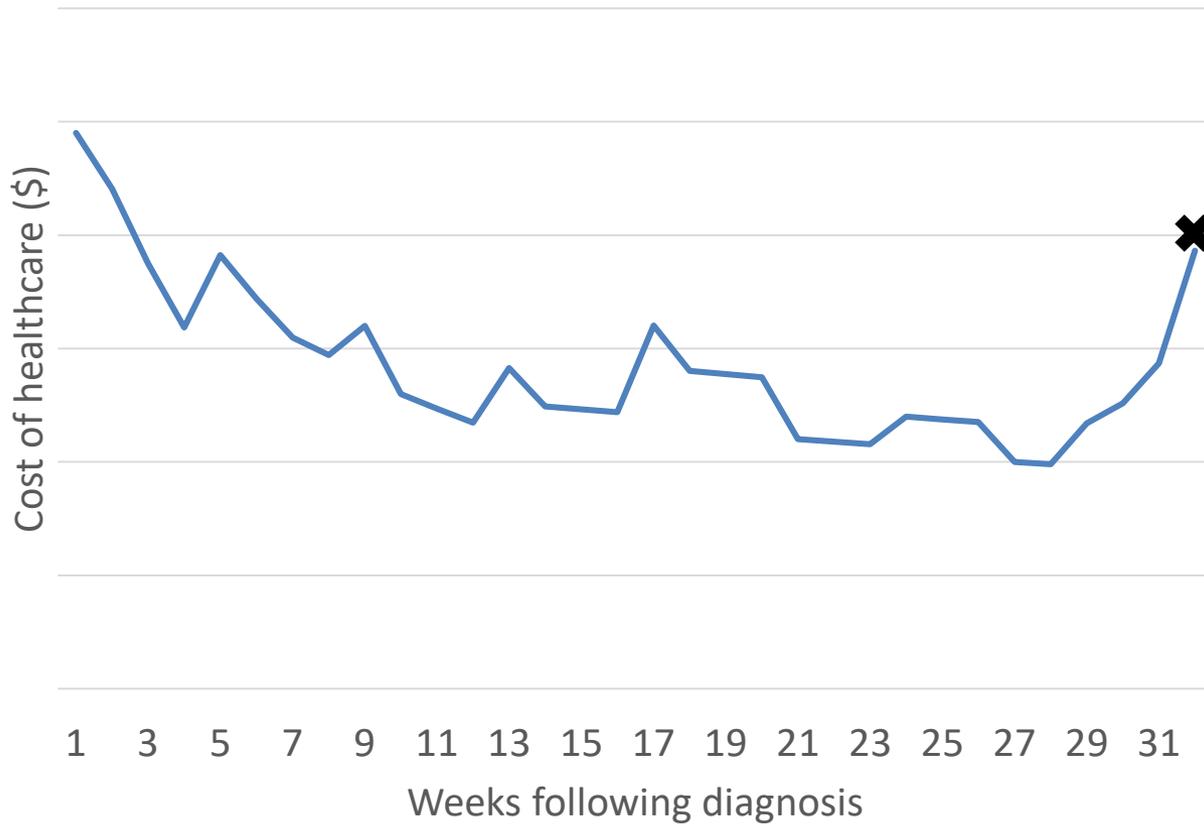
Data approximate to Temel/Greer reported outcomes but do not reflect specifics.

This is an illustrative exercise not a critical one.
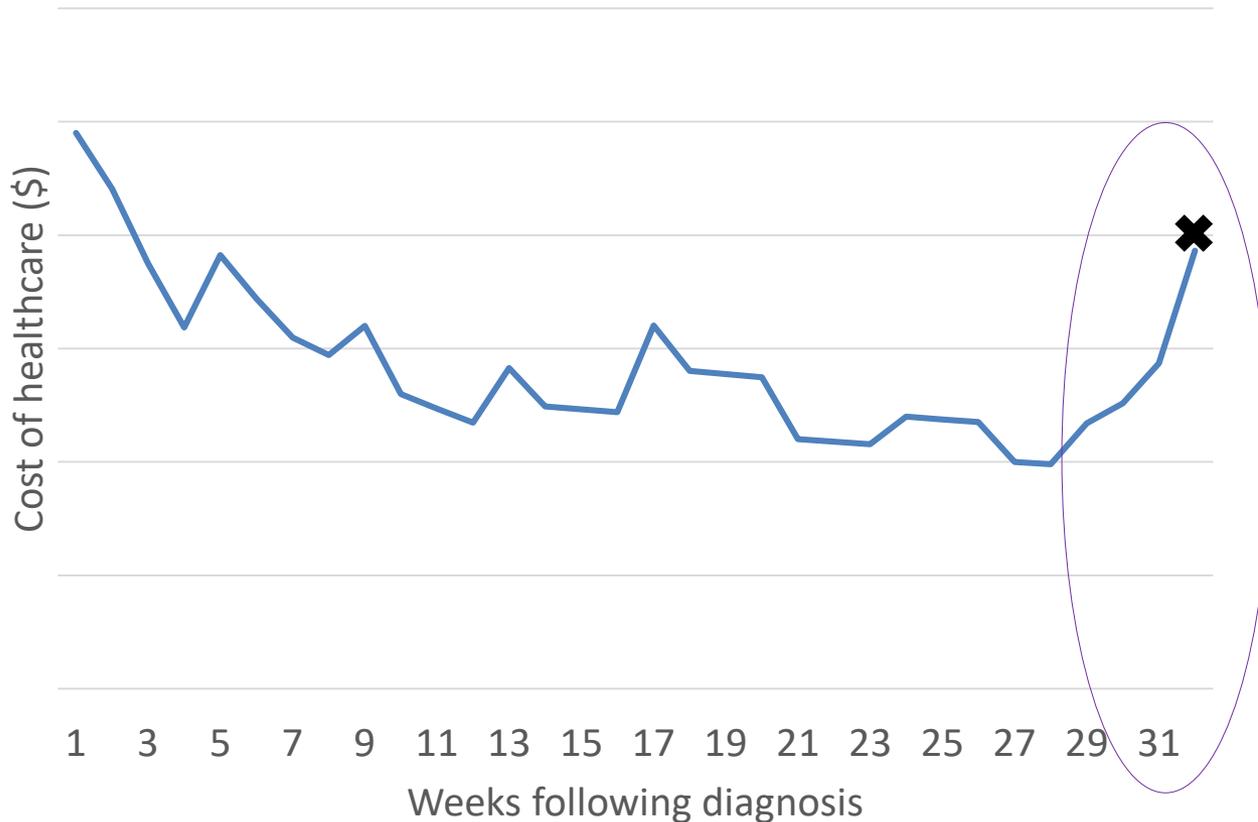
Current evidence

Usual care patient

UC patient:
- Lives ~8mths from diagnosis with spike in costs near end of life.

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Current evidence

Usual care patient

UC patient:
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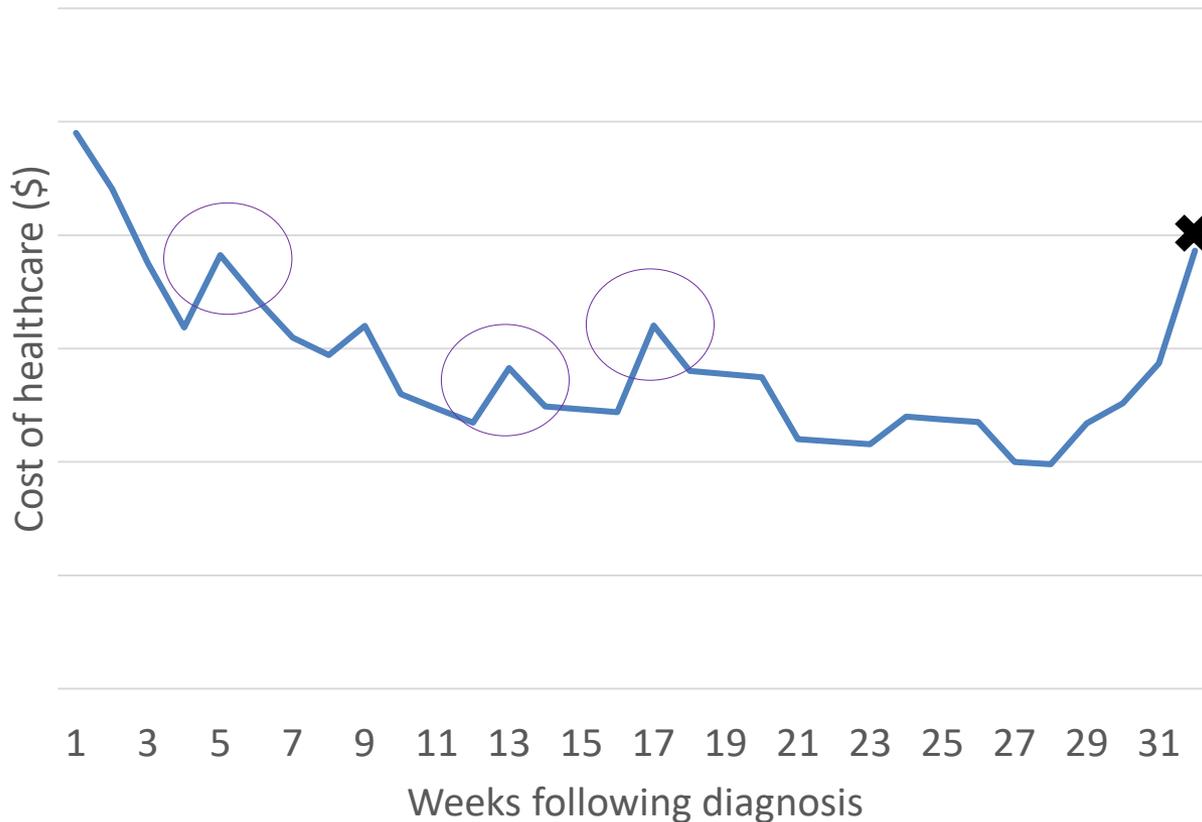
Current evidence

Usual care patient

UC patient:
- Lives ~8mths from diagnosis with spike in costs near end of life

- Has a jagged cost curve indicating episodic high-intensity treatment

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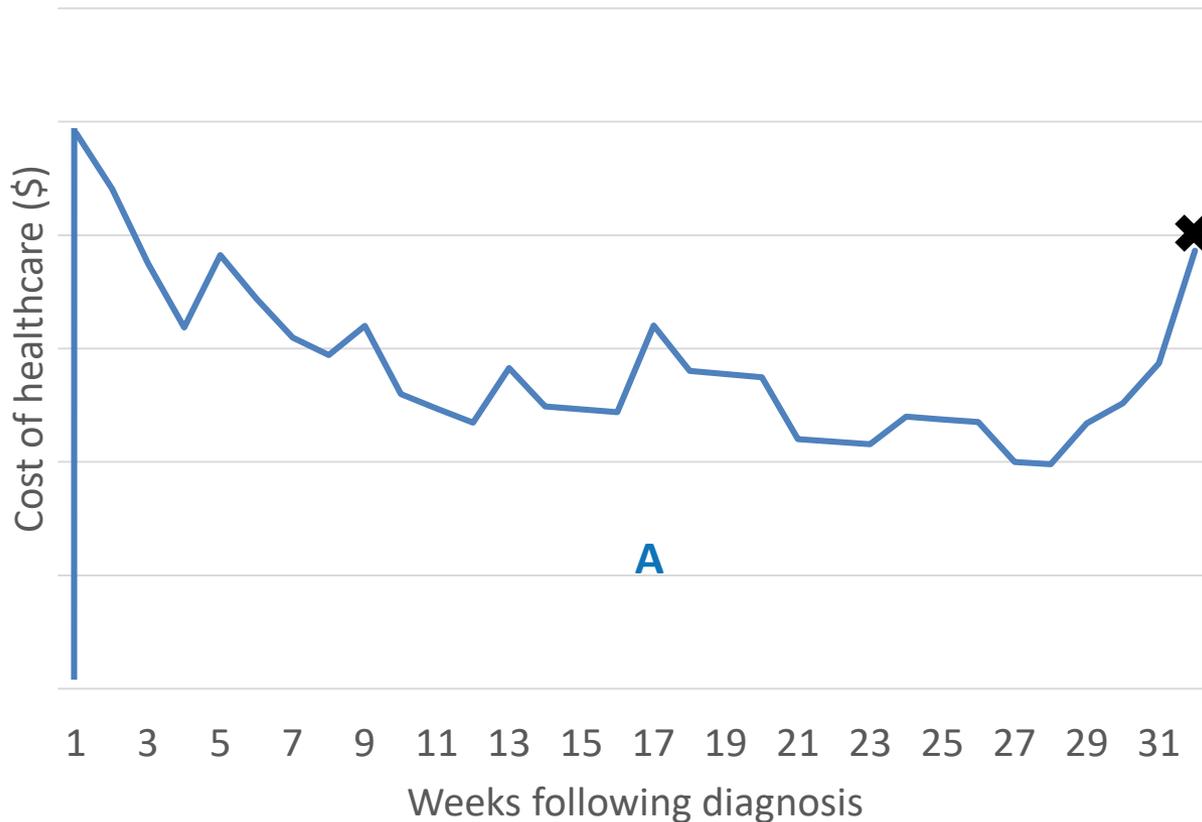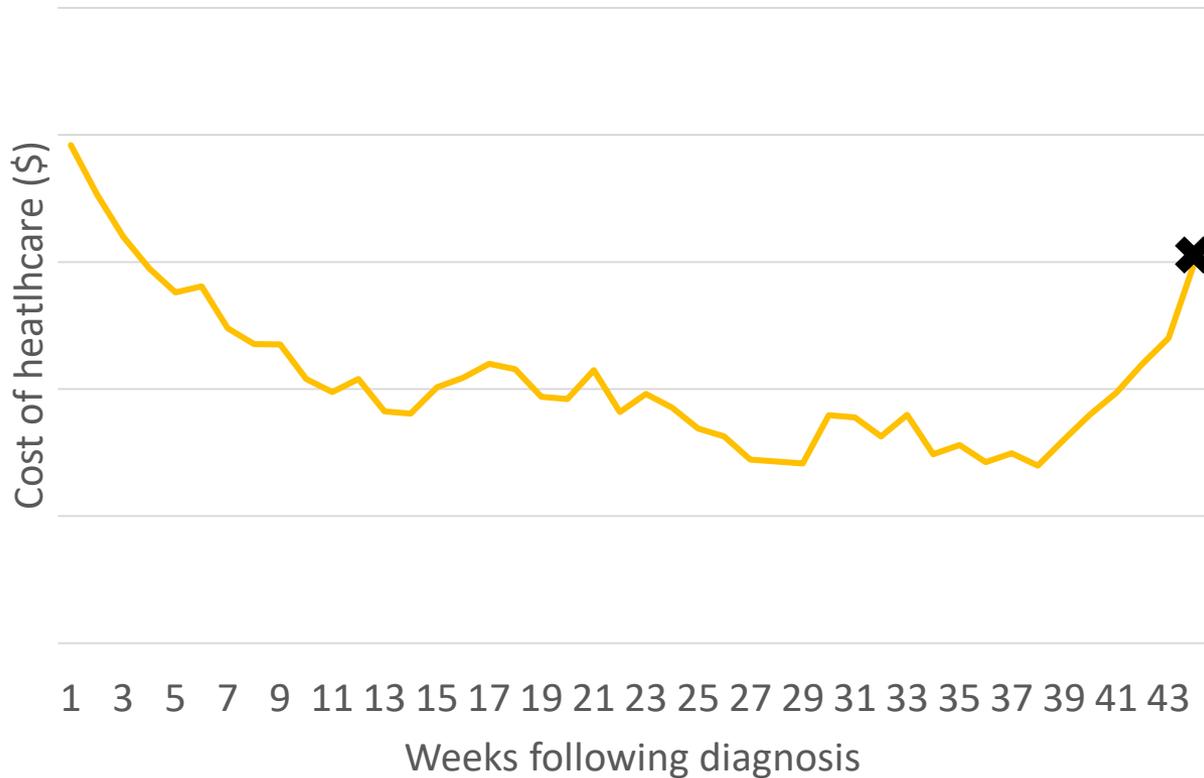
Current evidence

Usual care patient

UC patient:

- Lives ~8mths from diagnosis with spike in costs near end of life
- Has a jagged cost curve indicating episodic high-intensity treatment
- Accrues formal costs given by **A**, the area under this curve

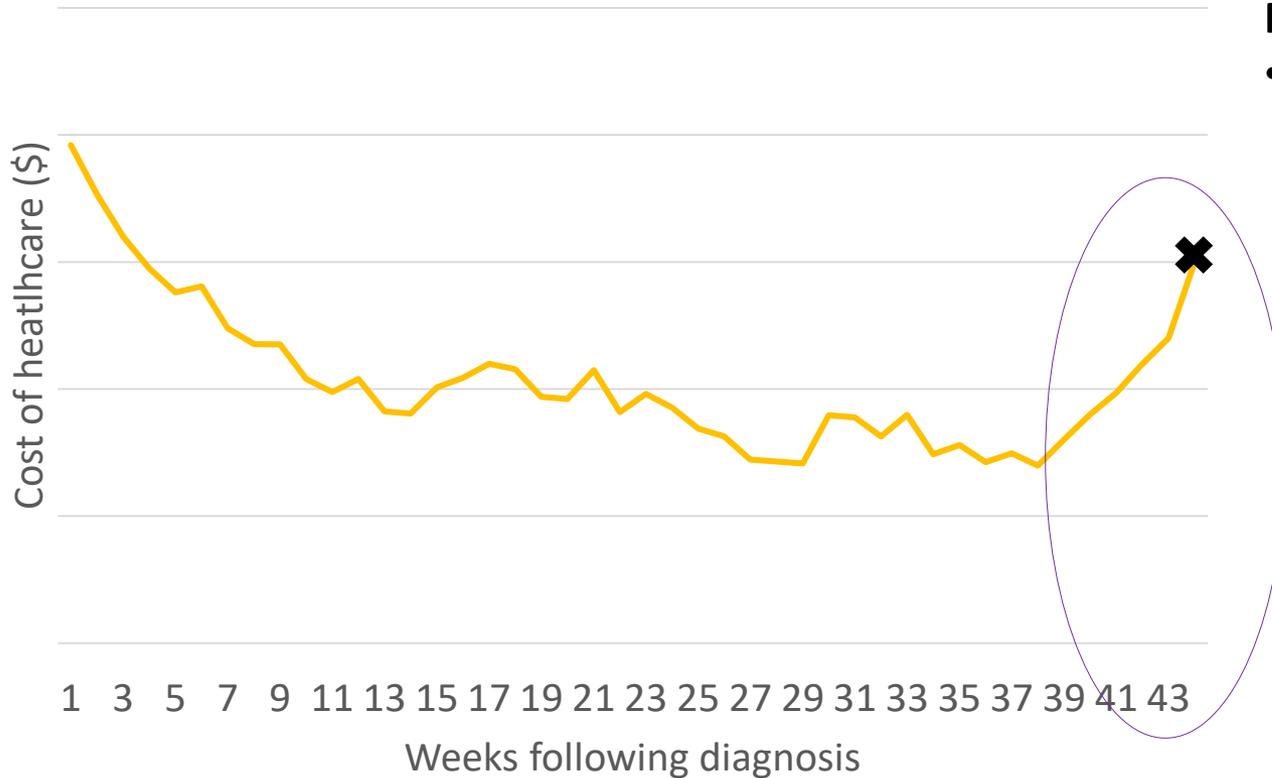# Current evidence

Palliative care patient

PC patient:
- Lives ~11mths from diagnosis with spike in costs near end of life

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Current evidence

Palliative care patient

PC patient:
- Lives ~11mths from diagnosis with spike in costs near end of life

Y-axis: Cost of heatlhcare ($)

X-axis: Weeks following diagnosis (1 3 5 7 9 11 13 15 17 19 21 23 25 27 29 31 33 35 37 39 41 43)

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Current evidence

Palliative care patient

PC patient:
- Lives ~11mths from diagnosis with spike in costs near end of life
- Has few 'peaks', i.e. a lack of intensive episodes

Current evidence

Palliative care patient

PC patient:
- Lives ~11mths from diagnosis with spike in costs near end of life
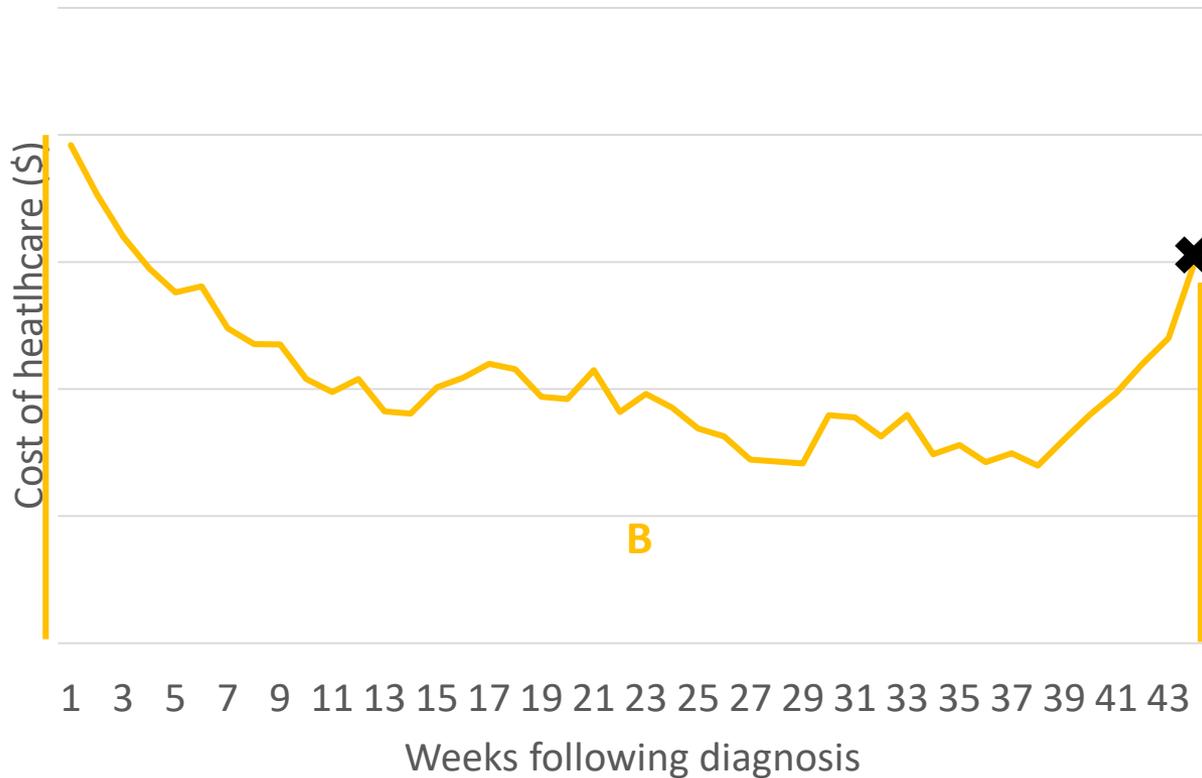- Has a jagged cost curve indicating episodic high-intensity treatment

- Accrues formal costs given by **B**, the area under this curve

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Current evidence

Observing a full episode of care

So, how is it possible for PC to:

- reduce initial intensity (weeks 1-12)

- reduce cost in the last 30 days of life

- increase costs overall?

Current evidence

Observing a full episode of care

So, how is it possible for PC to:

- **reduce initial intensity (weeks 1-12)**

- reduce cost in the last 30 days of life

- increase costs overall?

Current evidence

Observing a full episode of care

@12 weeks Temel (2010) reports less aggressive care for PC patients

PC cost reduction reflected in lower cost curve (difference in costs @ 12 weeks = area between the curves)

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Current evidence

Observing a full episode of care

So, how is it possible for PC to:

- reduce initial intensity (weeks 1-12)

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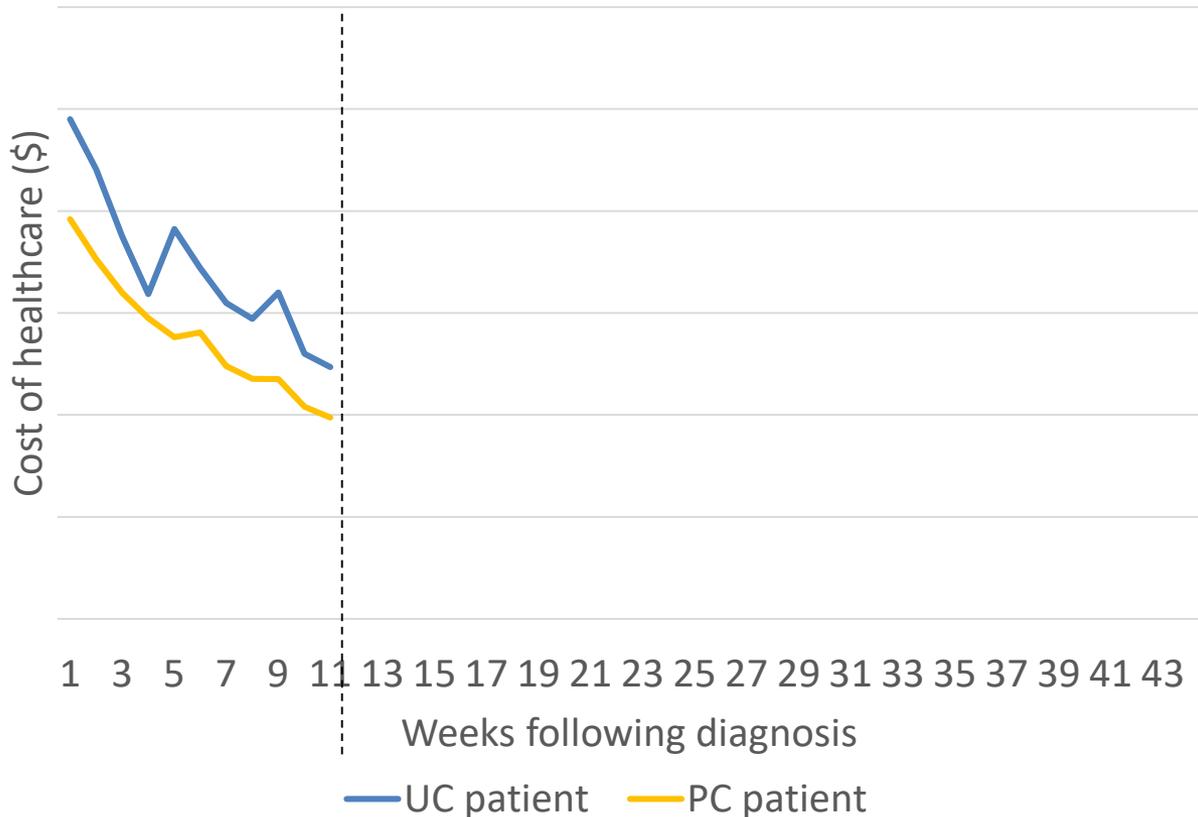
Current evidence

Observing a full episode of care

Greer (2016) reports less aggressive care for PC patients in last 30 days of life

PC cost reduction reflected in lower cost curve (difference in costs = area between the curves)

Cost of healthcare ($)

30 29 28 27 26 25 24 23 22 21 20 19 18 17 16 15 14 13 12 11 10 9 8 7 6 5 4 3 2 1
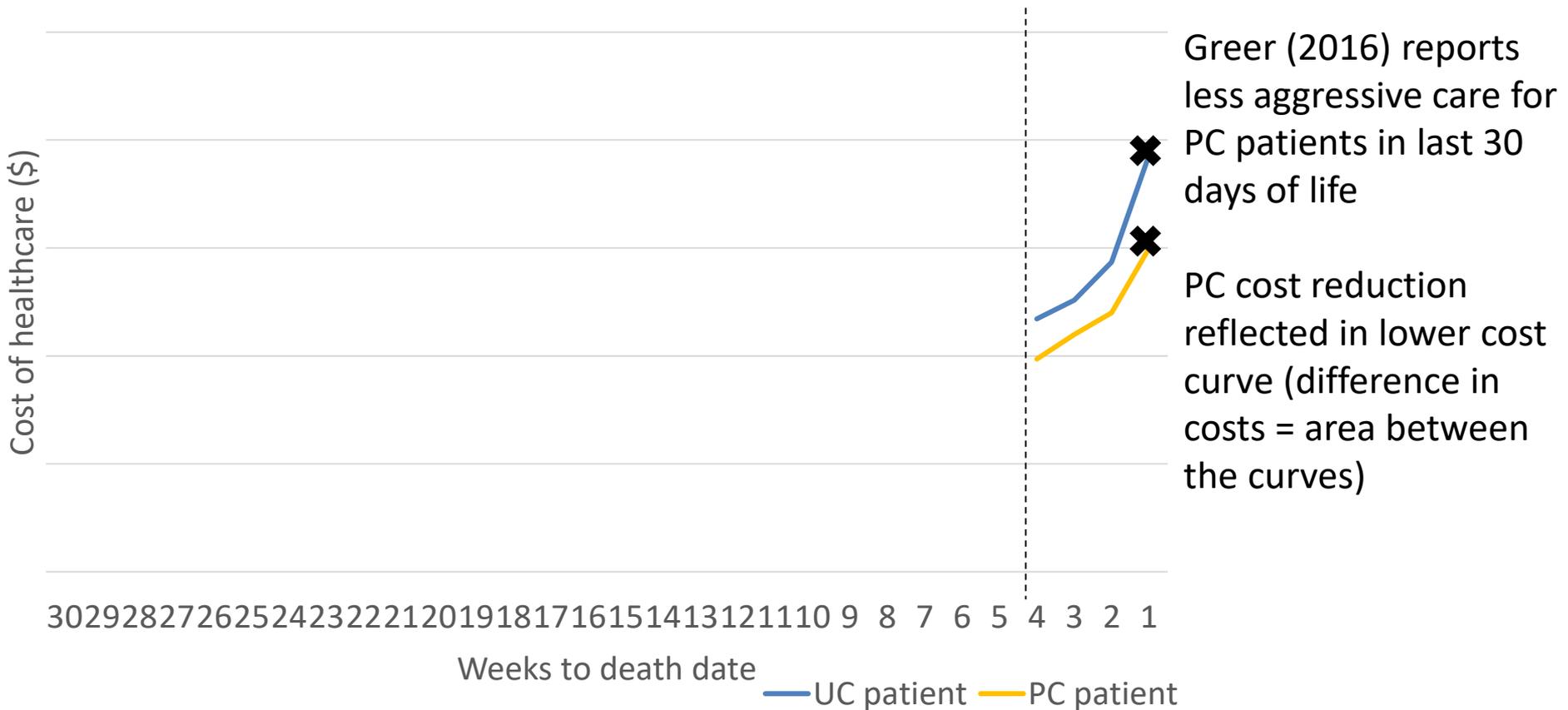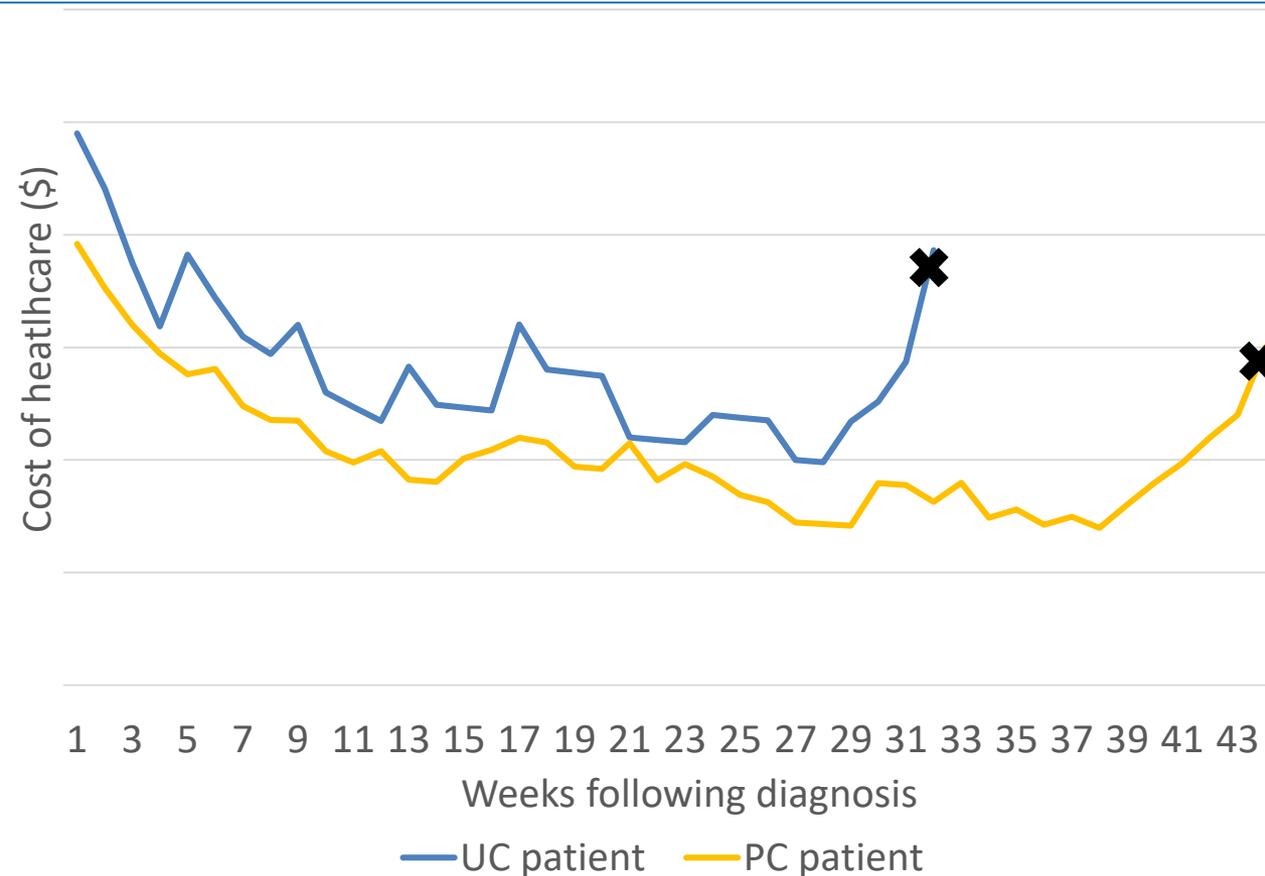
Weeks to death date

—— UC patient —— PC patient

So, how is it possible for PC to:

- reduce initial intensity (weeks 1-12)

- reduce cost in the last 30 days of life

- **increase costs overall?**

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Current evidence

Observing a full episode of care

Only when looking across the whole episode of care is the explanation apparent:

Cost of heatlhcare ($)

Weeks following diagnosis

—UC patient   —PC patient

Current evidence

Observing a full episode of care

Only when looking across the whole episode of care is the explanation apparent:

- **PC was less intensive and so lower cost for ~8mths following diagnosis** (shown by the area, **X**, between the two curves)

Current evidence

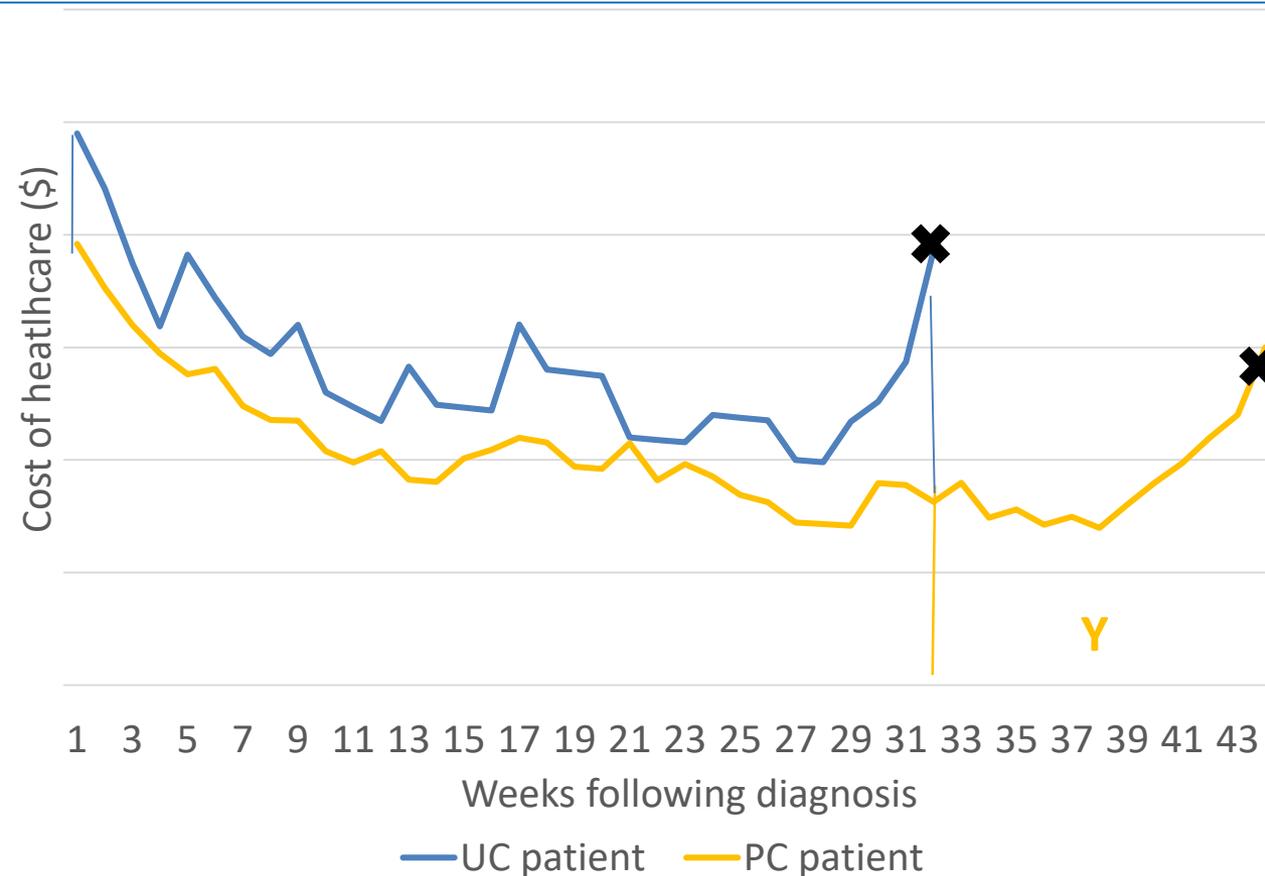Observing a full episode of care

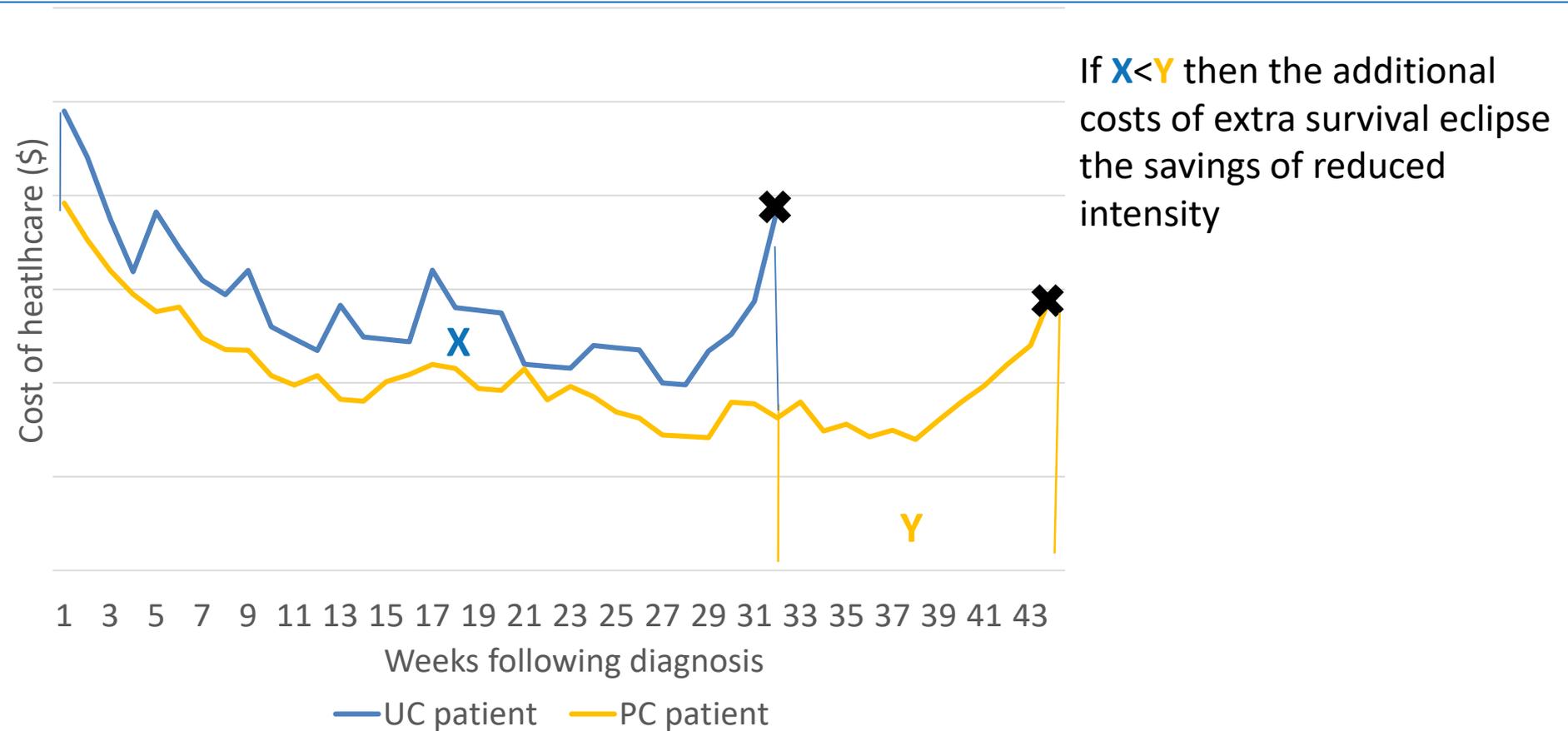Only when looking across the whole episode of care is the explanation apparent:

- PC was less intensive and so lower cost for 6+ months following diagnosis

- **PC patient lived an additional three months and accrued further costs, denoted by area Y**

Current evidence

Observing a full episode of care

If **X**<**Y** then the additional costs of extra survival eclipse the savings of reduced intensity
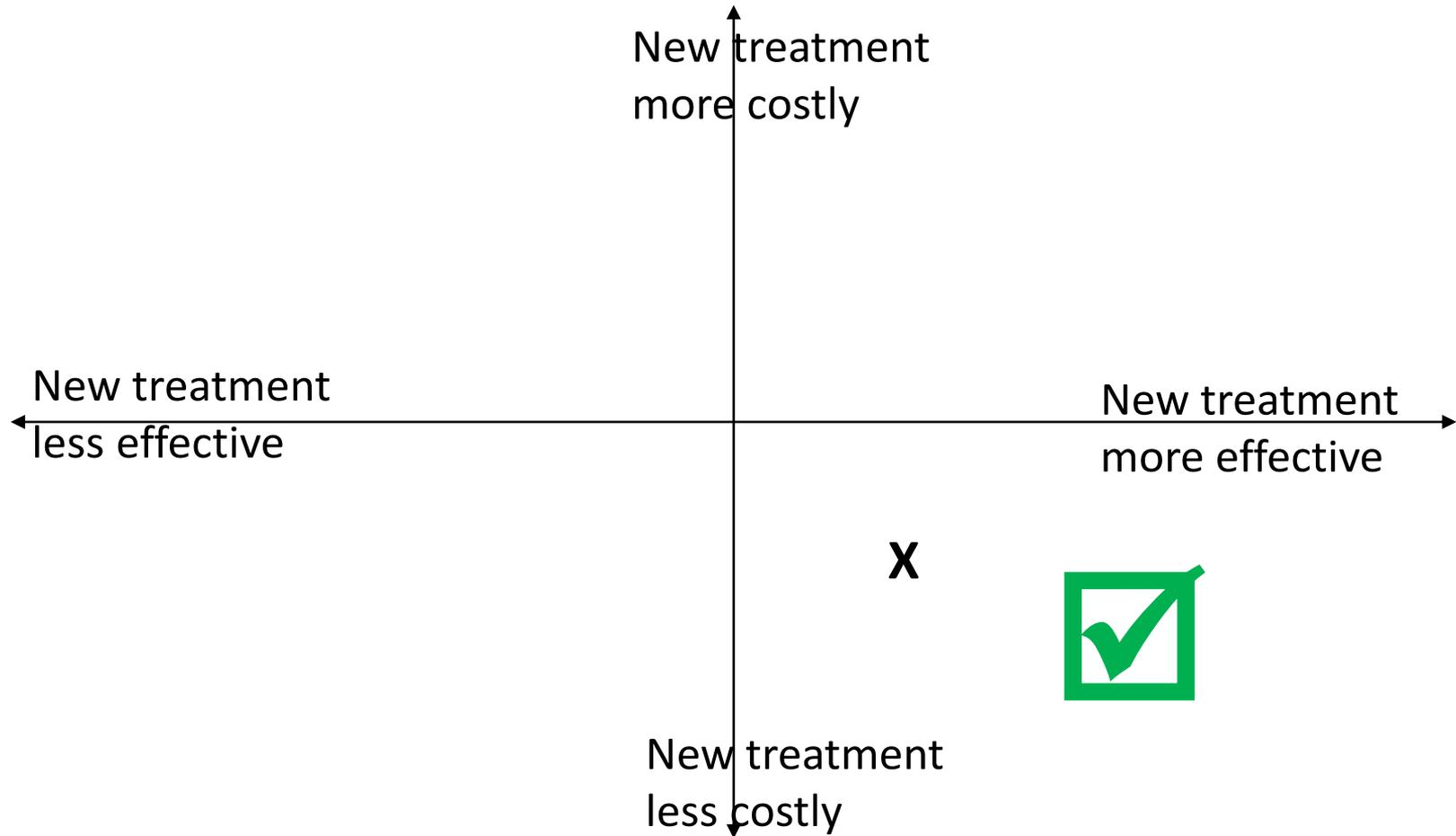
Current evidence

Important note

- This **does not** mean that we think that an intervention with substantial survival effects is not worthwhile

- Only that it likely won't be associated with any cost-saving

- This is well understood by 'fiscal' economists, not always in health
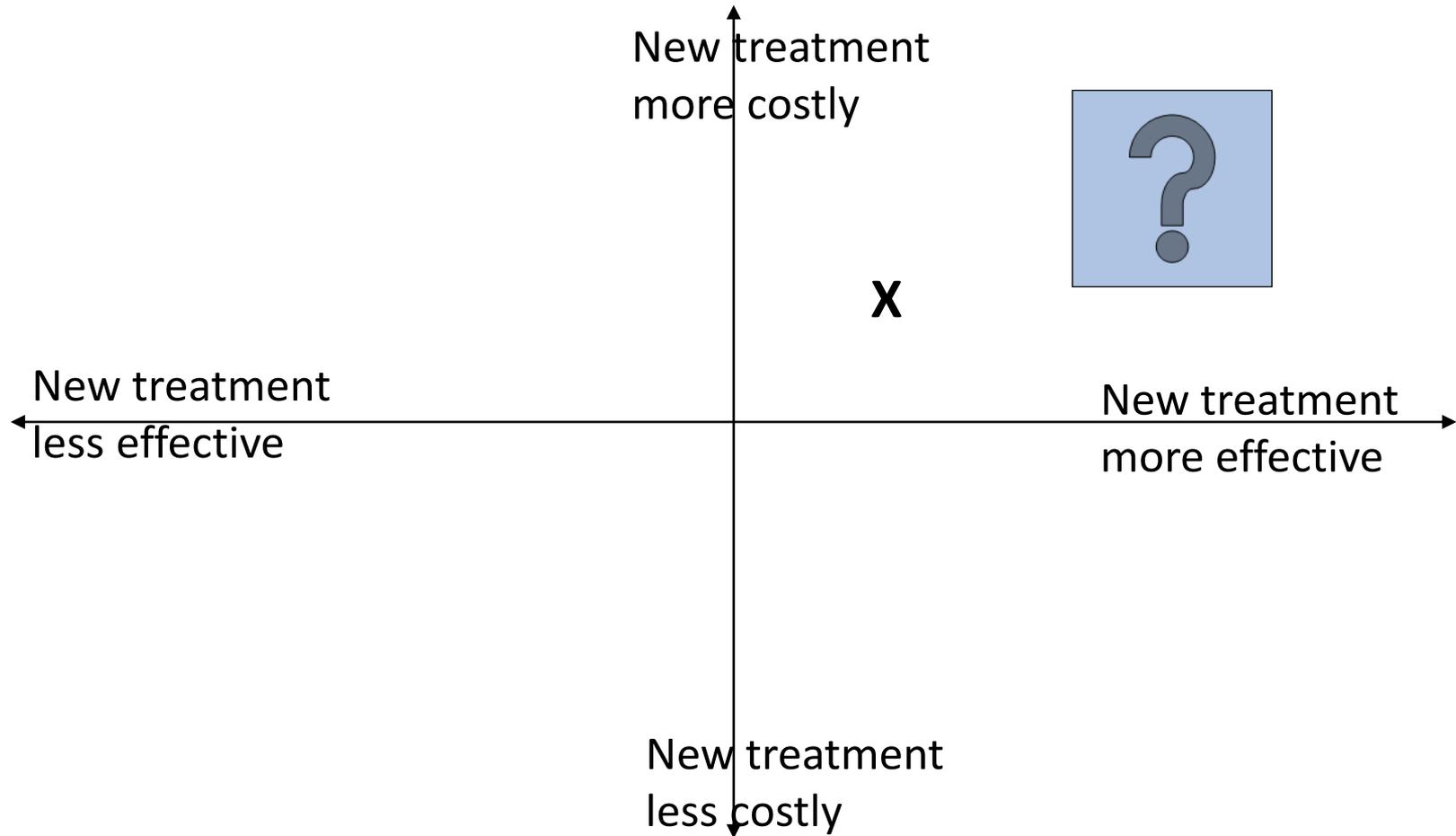
Current evidence

Cost-consequence analysis

Current evidence

Cost-consequence analysis

New treatment
more costly

New treatment
less effective

X

New treatment
more effective

New treatment
less costly

Current evidence

Limitation (iii): Perspective

- <u>Whose</u> costs?

  - Hospital studies focus on hospital costs

  - Charges studies focus on payer (e.g. Medicare) costs

  - Out-of-pocket and informal costs comparatively ignored

    - ➤ Risk that observed cost-savings are passed on to other parts of the system or to patients and families

    - ➤ Similar issues to survival example – partial viewpoints distort reality

Current evidence

Limitation (iv): Intervention timing and what is "palliative care" anyway?

- Earlier intervention (I) has a larger effect on hospital costs

  ➢ Timing must be incorporated or bias to the null

- But how?

  ▪ Currently I within t days of admission

    ○ No clinical guidelines to define t; outliers a problem

  ▪ Optimally a continuous variable

    ○ Typical dose response assumes normal distribution

    ○ Skewed exposure and outcome xvars

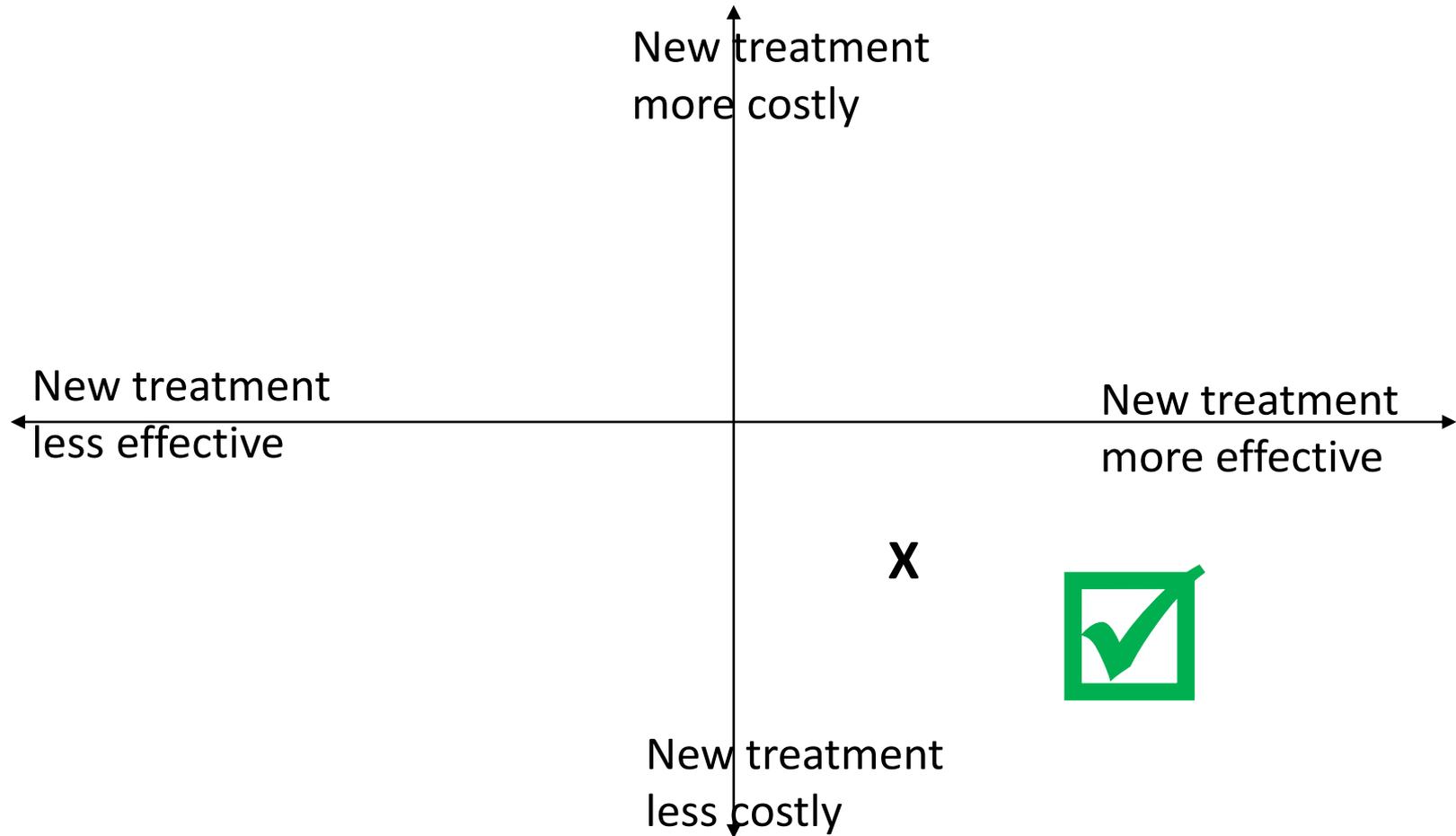   ○ More complex still across the disease trajectory!

Summary

- Evidence on **<u>cost</u>** of care for medical complexity is unarguable: costs are high and going higher (particularly in the US)

- Evidence on **<u>PC effect</u>** on these costs sometimes reported as unarguable ("PC saves money") but reality more complicated

- Studies to date have limitations that may lead to overestimation

- Limitations not arbitrary; reflect routine data collection

- Critical for long-term development of policy and services that limits are addressed through expanded scope

- Even if not studying costs, do bear in mind questions
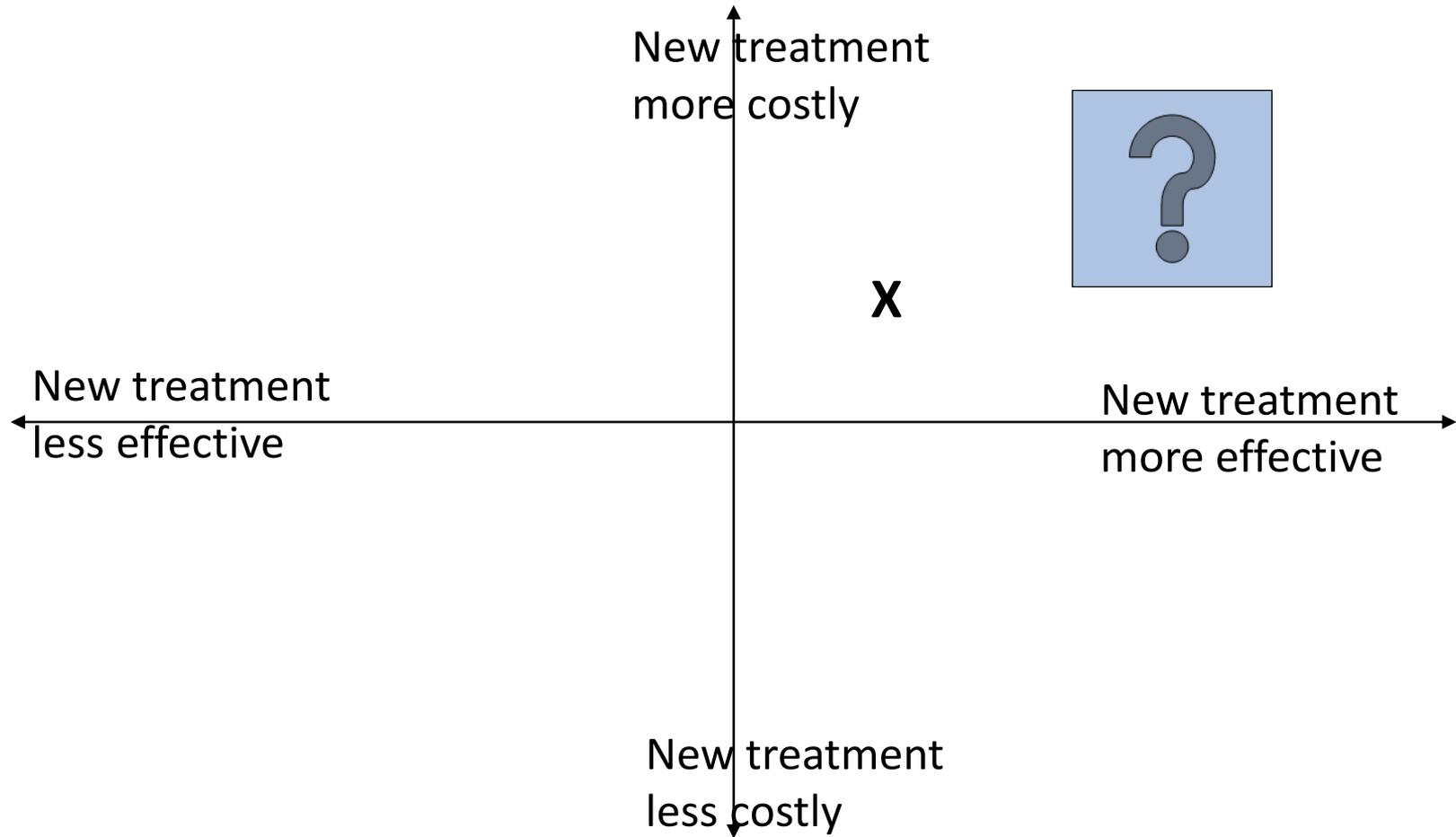
  - <u>What, when, for whom?</u>

Summary

One interpretation of current literature

Summary

An alternative we should be ready for

Overview

Part 1: Conceptual issues (May)

- Health economic evaluation: what and why?

- Economic evaluation and palliative care

Part 2: Key issues in the evidence base (Aldridge)

- Dying in America study

- Group presentations of key articles

Part 3: Practical considerations (May)

- Economic evidence on palliative care

- Practical considerations in conducting a study

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Defining a research question

What, when, for whom?

- An economic research question will compare the costs (and consequences) of two options

- Most in the literature are broad, e.g.

  - What is the effect of palliative care on costs compared to usual care for adults with serious illness?

- Recent evidence recommends more detailed questions:

  - Intervention

  - Outcome

  - Target population

Defining a research question

- Consider intervention **timing**:

  - Earlier intervention more effective for hospital admissions (May & Normand, 2016) and LYOL (Scibetta et al., 2016)

- Consider outcome **perspective**:

  - PC reduces hospital costs (but CMS costs? Family costs?)

- In both cases, widest view is the best (and the hardest to achieve)

Defining a research question

Advice

- Consider target **population**:

  - What is the effect of palliative care on costs compared to usual care **for adults with serious illness**?

- Early studies assume treatment effect homogeneity but evidence of great heterogeneity (May et al., 2018):

  - PCC cost-effects larger for cancer & for more comorbidities

  ➢ Research populations who are particularly complex and/or understudied (e.g. dementia, multimorbidity)

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Statistical model

Awkwardness of healthcare utilization data

Distributions typically pose problems for statistical analysis:

- **Non-negativity:** by definition never less than zero

- **Mass of zero-value observations:** in data drawn from populations, a large number of cost data-points will be zero

- **Positive skew:** a minority of patients incur a disproportionately high level of costs, skewing the distribution right

- **Heteroscedasticity:** variability of costs is unequal across a range of values for important predictors

- **Leptokurtosis:** clustering of cost observations for a large number of patients with similar care trajectories may result in high 'peaked-ness' of distribution
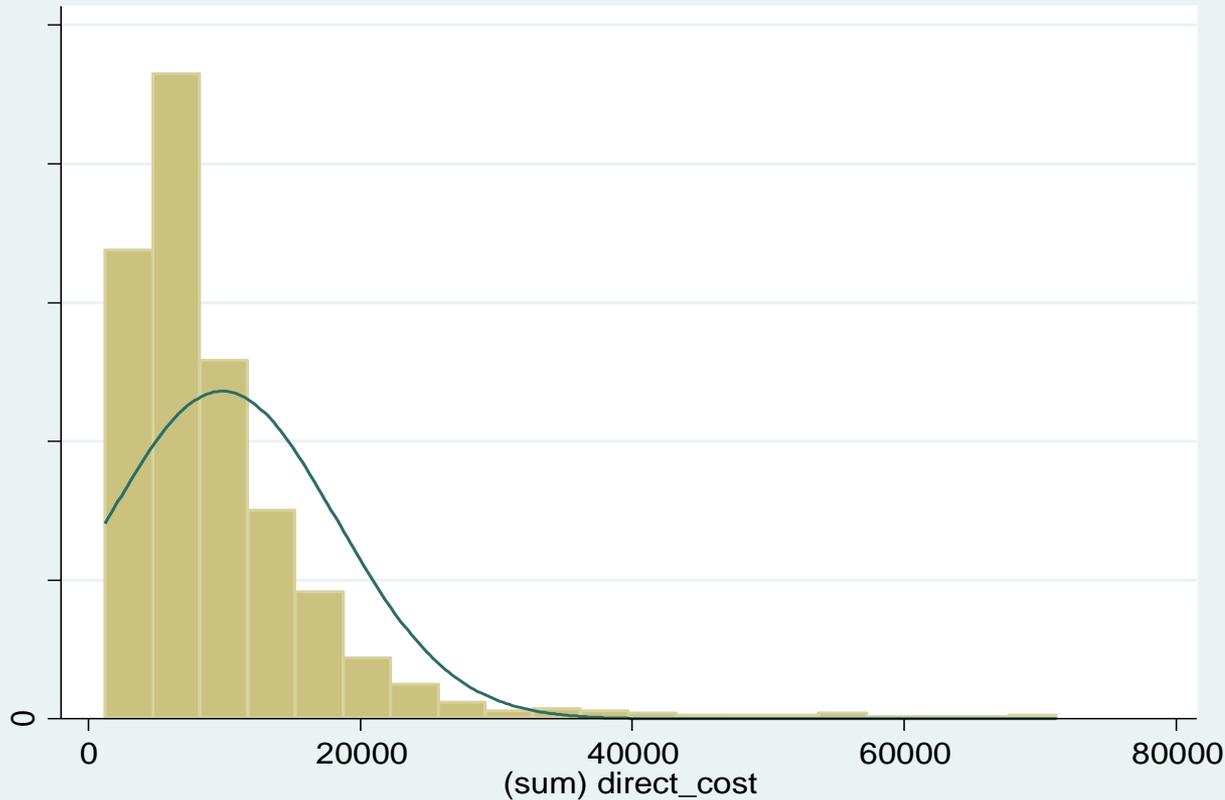
➢ **<u>Linear regression (OLS) is seldom appropriate</u>**

Statistical model

Awkwardness of healthcare utilization data

Total direct cost of hospital admission

Skewness: 3.2

(0 for normal distribution)

Kurtosis: 17.7

(3 for normal distribution)

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Statistical model

The 'old' way to address this was log-transformation, which generally mitigates skew, heteroscedasticity & leptokurtosis

ln(total direct cost) of hospital admission

Skewness: 0.4

   (0 for normal distribution)

Kurtosis: 3.4

   (3 for normal distribution)

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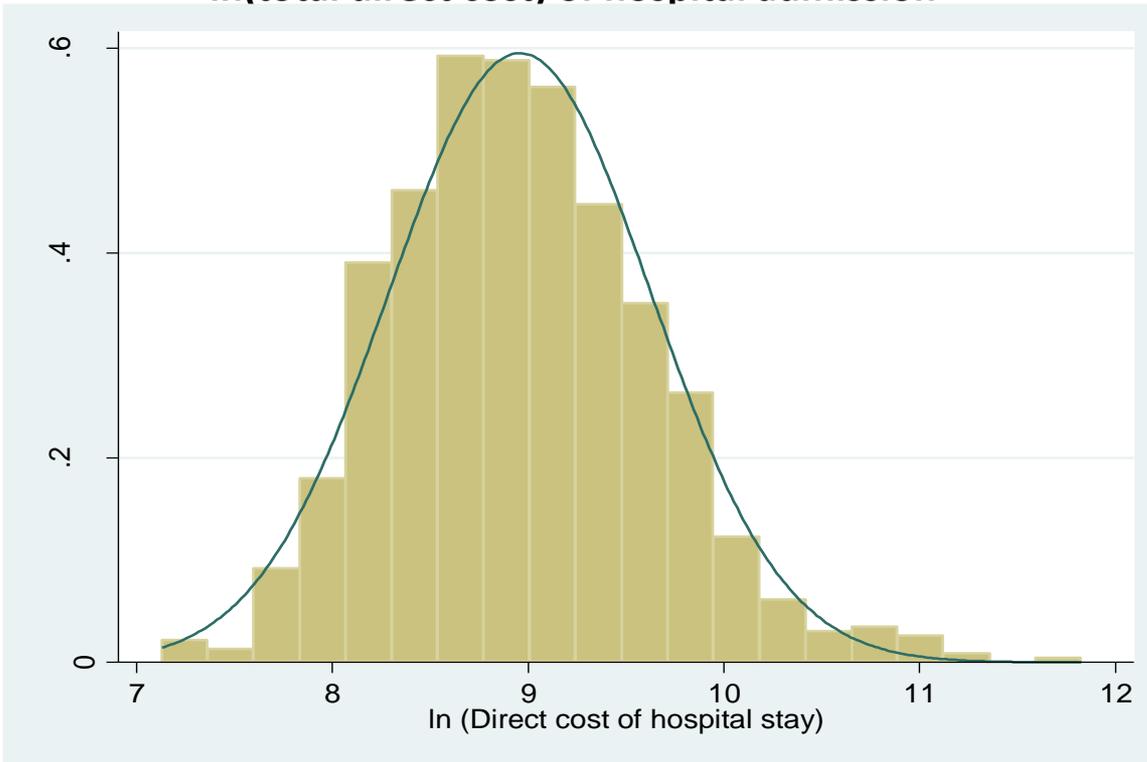Coláiste na Tríonóide, Baile Átha Cliath
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Statistical model

However, beware the 'retransformation problem':

"Although [log-transformed] estimates may be more precise and robust [than estimates using highly skewed distributions of untransformed costs], no one is interested in log model results on the log scale per se.

"Congress does not appropriate log dollars. First Bank will not cash a check for log dollars. Instead, the log scale results must be retransformed to the original scale so that one can comment on the average or total response to a covariate x.

"There is a very real danger that the log scale results may provide a very misleading, incomplete, and biased estimate of the impact of covariates on the untransformed scale, which is usually the scale of ultimate interest." - Manning (1998)

Statistical model
Awkwardness of healthcare utilization data

Consider instead non-linear alternatives to OLS:

Generalized linear model

Family	Link
Gaussian	Identity
Poisson	Log
Gamma	Power
Inverse Gaussian	

Statistical model

Consider instead non-linear alternatives to OLS:

Generalized linear model

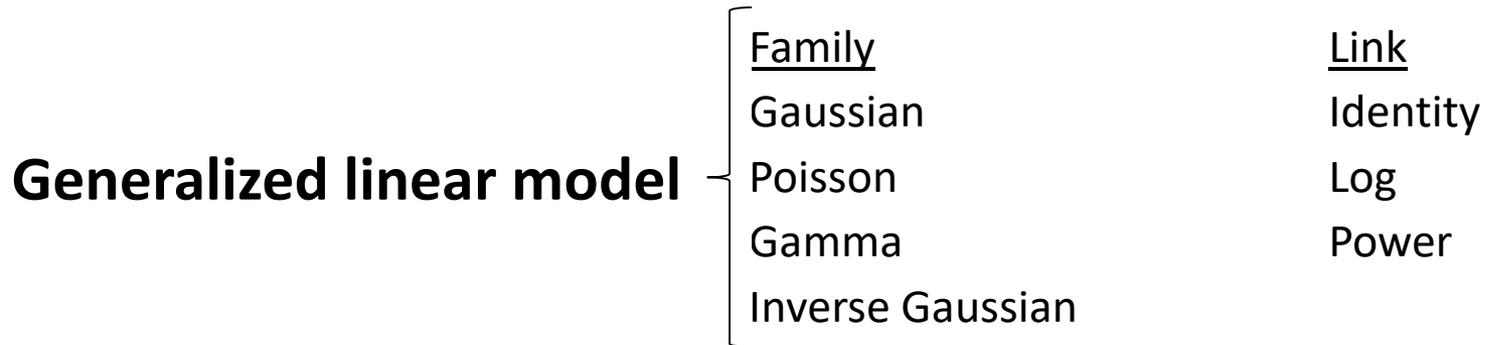| Family	Link
Gaussian	Identity
Poisson	**Log**
Gamma	Power
Inverse Gaussian	

Statistical model
Awkwardness of healthcare utilization data

Consider instead non-linear alternatives to OLS:

Generalized linear model

Family	Link
Gaussian	Identity
Poisson	Log
Gamma	Power
Inverse Gaussian	

Exponential conditional mean models

Generalized gamma models

Extended estimation equations

Finite mixture models

Statistical model
Awkwardness of healthcare utilization data

Stata programs available online to evaluate model performance:

- For GLMs only, Stata *glmdiag.do* from UPenn (http://www.uphs.upenn.edu/dgimhsr/stat-cstanal.htm)

- For all models, Stata *AHE_2ed_Ch_3&12.do* from University of York (http://www.york.ac.uk/economics/postgrad/herc/hedg/software/)

- These test the appropriateness of specific models to a given distribution
- No model is dominant
  - ➤ Evaluating models prior to analysis is essential to maximize accuracy of estimated effects

Statistical model

Advice

- Consider and describe data carefully prior to analysis

- Avoid use of OLS, OLS ln(y) and ANOVA with healthcare utilization data

- Consider nonlinear alternatives

  - Use available software to understand and evaluate options

  - Report briefly this process in Methods

Further reading:

- *The York .do file accompanies a book: Jones et al. (2013a)*

- *For an overview of why model choice matters, see Jones (2010)*

- *For more technical analyses, see Jones et al. (2013b); Garrido et al. (2012)*

- *Not my true expertise but I am happy to help if I can (peter.may@tcd.ie)*

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Additional considerations

- Do not remove outliers, e.g. define your sample by length of stay, match by length of stay, or use length of stay as a regression variable (May et al., 2016)

- If your cost data come from more than one year adjust for inflation using Consumer Price Index

- If your cost data come from more than one state adjust for cost of living using Medicare Wage Index
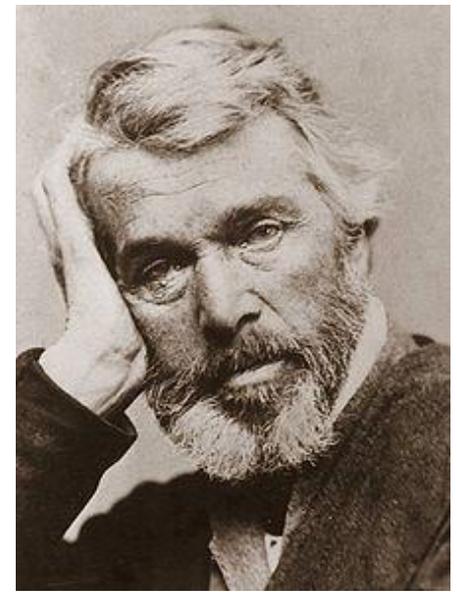
Summary

- Economics of palliative care studies require consideration re:

  - Intervention timing

  - Cost perspective

  - Target population

    ➤ Status quo reflects where data are routinely collected

    ➤ Priority is expanding scope, i.e. well-funded 1ary research or better linking existing data (Kelley et al., 2014; Maetens et al., 2016)

- Awkward data preclude use of ordinary regression

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Final thought

Thomas Carlyle (1795-1881) called economics 'the dismal science'

Economists might argue that it is **reality** that is dismal

Rationing inevitable in all health systems; economics merely a decision tool to navigate hard (often unpalatable) choices

Projections of health status and costs make it critical to both improve outcomes and control cost of care to seriously-ill people

➢ An opportunity to make a difference!

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Thank You

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