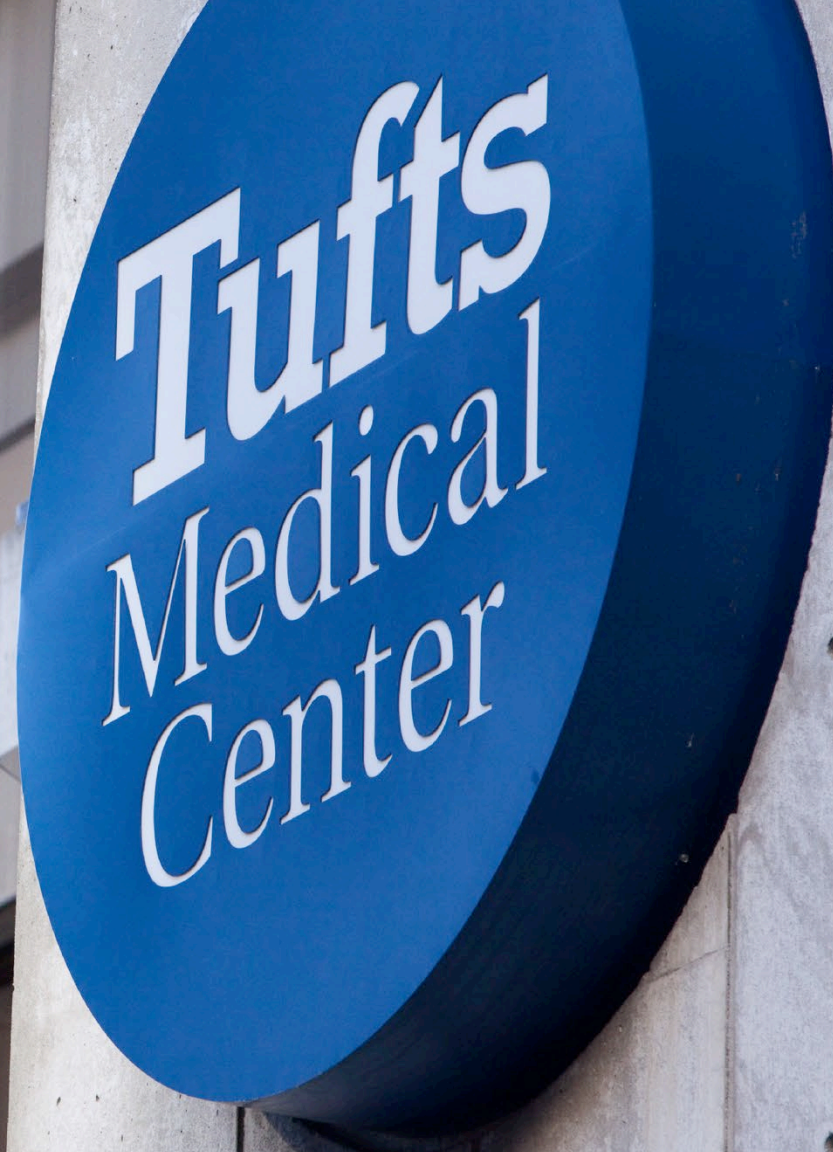


# The Right Price: A Value-Based Prescription for Drug Costs


VA Health Economic Resource Center's  
Health Economics Cyber Seminar Series

Peter J. Neumann, ScD & David D. Kim, PhD  
March 16, 2022

Always  
Thinking  
Ahead.™



# New Cancer Treatments Top \$500,000 And Raise Daunting Questions About How To Pay

January 11, 2018 By [Richard Knox](#) 

## THE WALL STREET JOURNAL.

World U.S. Politics Economy **Business** Tech Markets Opinion Life&Arts RealEstate WSJ.Magazine

### The Million-Dollar Cancer Treatment: Who Will Pay?

So far, few patients have received the new drugs, as commercial health plans and Medicare wrestle with how to cover the treatment

By [Jonathan D. Rockoff](#)  
April 26, 2018 7:00 a.m. ET

BIOTECH AND PHARMA

## FDA approves Novartis' \$2.1 million gene therapy — making it the world's most expensive drug

PUBLISHED FRI, MAY 24 2019 • 1:03 PM EDT | UPDATED FRI, MAY 24 2019 • 3:11 PM EDT

The New York Times

 **TheUpshot**

### *New Drug Could Cost the Government as Much as It Spends on NASA*

The Alzheimer's treatment will cost \$56,000 per patient, and millions may use it. The result: "crazy numbers" for Medicare.

        425



By [Josh Katz](#), [Sarah Kliff](#) and [Margot Sanger-Katz](#)

Published June 22, 2021 Updated June 23, 2021

# THE RIGHT PRICE

A Value-Based Prescription  
for Drug Costs



Peter J. Neumann | Joshua T. Cohen | Daniel A. Ollendorf

# Overview

- Why write the book?
- Part 1: The Economics of Prescription Drugs
- Part 2: Experiences Measuring a Drug's Value
- Part 3: Capturing Broader Value Elements
- Part 4: Getting to Value-Based Drug Prices

# Why write the book?

- Make the case for *value-based* drug prices
- Fill a void
- A book provides a special opportunity

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# The Prescription Drug Market



# The Prescription Drug Market

- The “demand” side
- The “supply” side
- Most proposed solutions will not align price with value



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# Measuring value: Outside the US

- Formal health technology assessment
  - Does clinical evidence support adoption?
  - Is investment worth the price?
- Cost/QALY countries (e.g., UK)
- Non-cost/QALY countries (e.g., Germany)

# Measuring value: In the US

- Mistrust of central HTA
- Distaste for “rationing”
- Limited and isolated efforts

**A new era?**



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**The New York Times**

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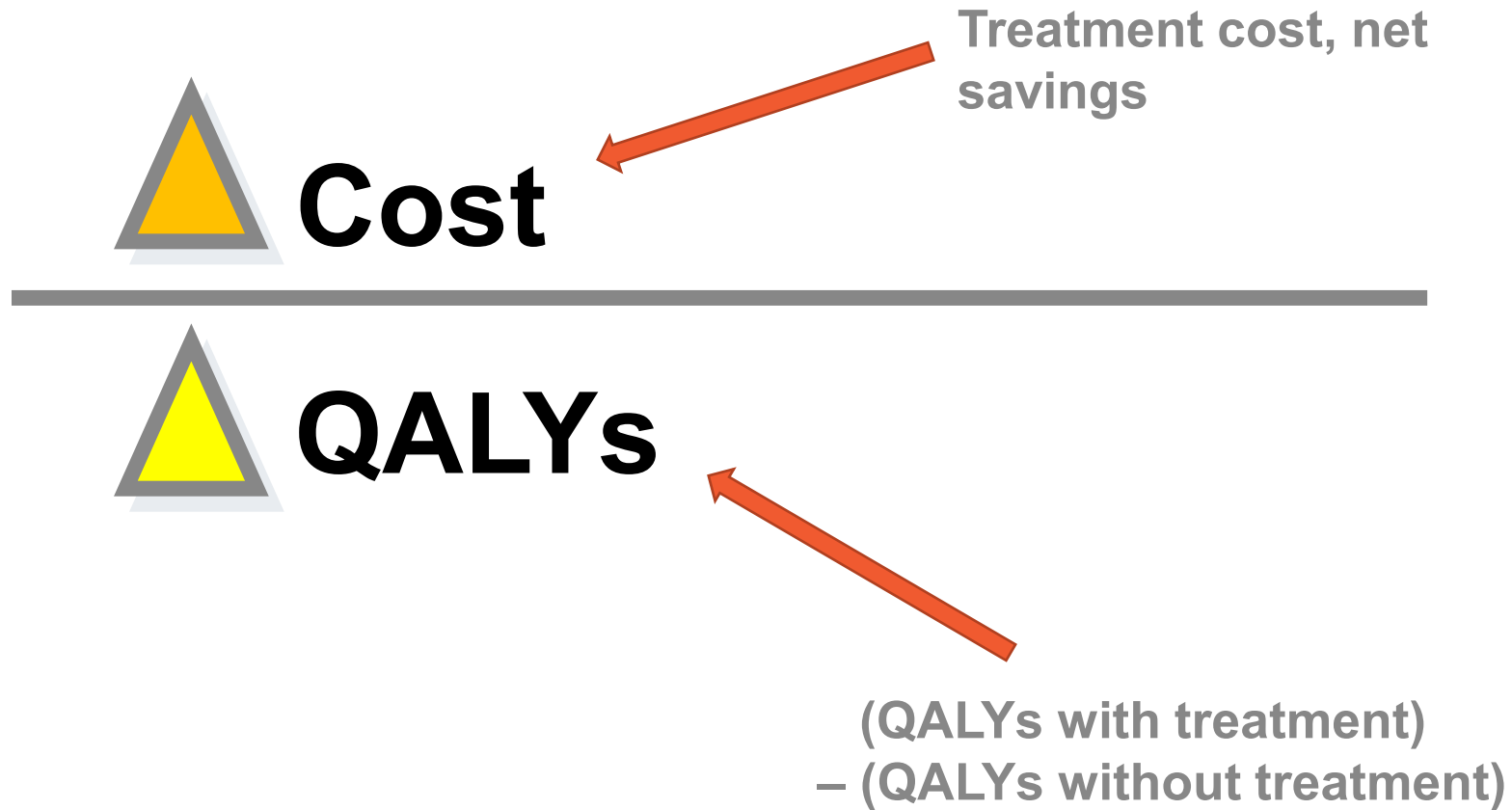
# A Drug Costs \$272,000 a Year. Not So Fast, Says New York State.

New York's Medicaid program says Orkambi, a new drug to treat cystic fibrosis, is not worth the price. The case is being closely watched around the country.

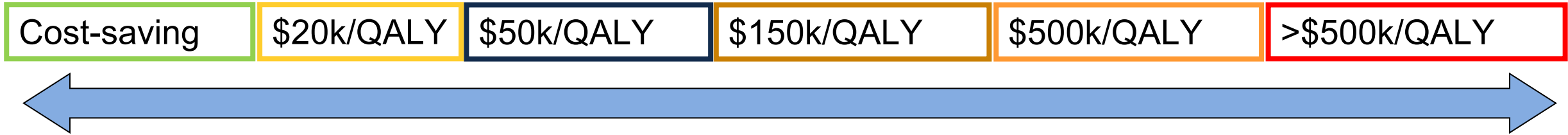
**By Katie Thomas**

June 24, 2018

# COST/QALY RATIO



# Selected recent ICER reviews



Modulator therapies for cystic fibrosis

Zolgensma for SMA

Luxturna™ for inherited retinal disease

CAR-T therapy for B-Cell cancers

Emicizumab for Hemophilia A

# Poll question #1

- **What is the most appropriate cost-effectiveness threshold in the US?**
  - \$50,000/QALY
  - \$100,000/QALY
  - \$150,000/QALY
  - \$200,000/QALY
  - Above \$200,000/QALY



# Criticisms of ICER

- Lack of accountability
- Doesn't reflect patient and payers needs
- “One-size-fits-all approach”
- Unease with QALYs
- Excludes important societal elements of value

# Overview

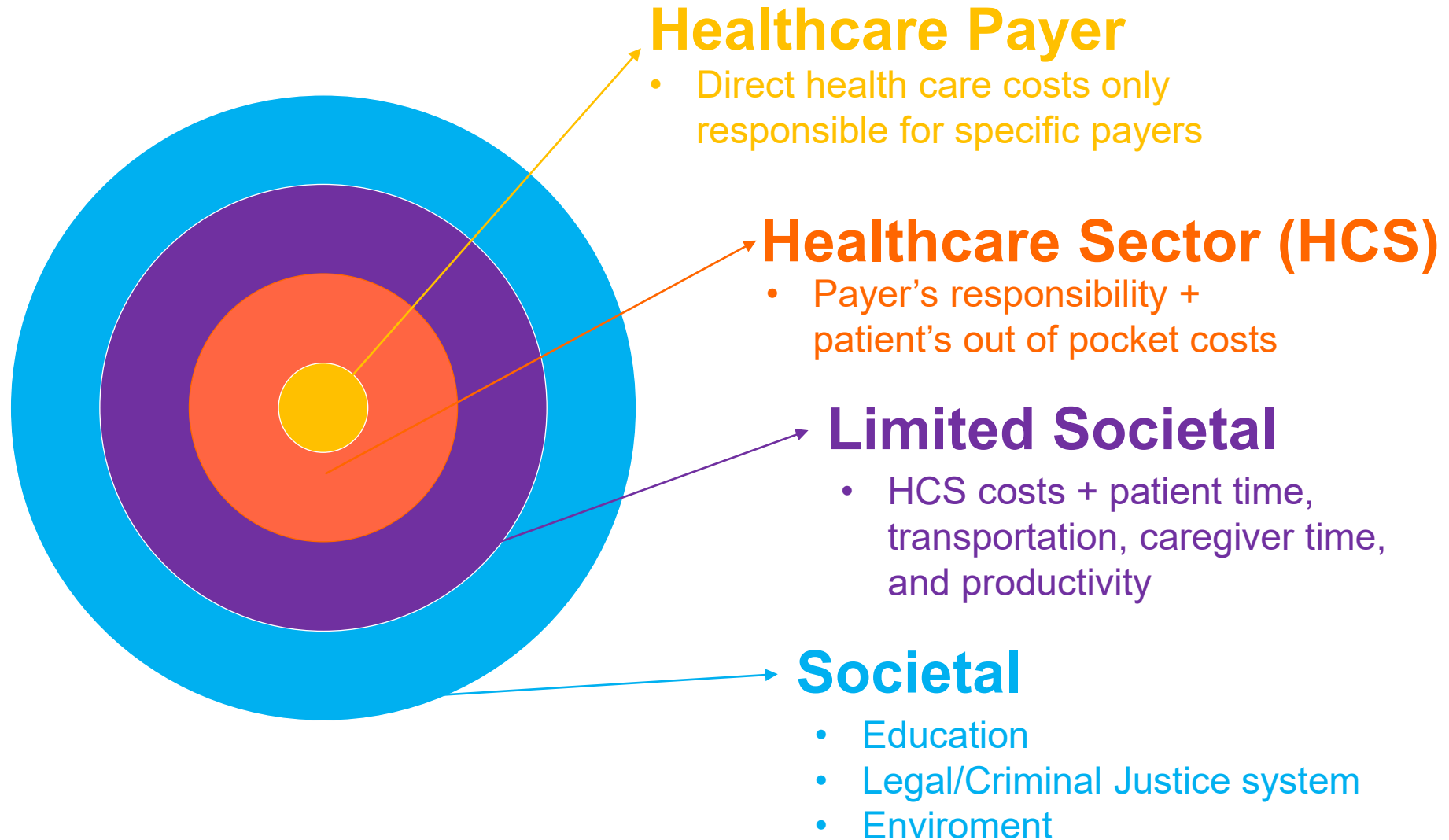
- Why write the book?
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# Perspective matters!

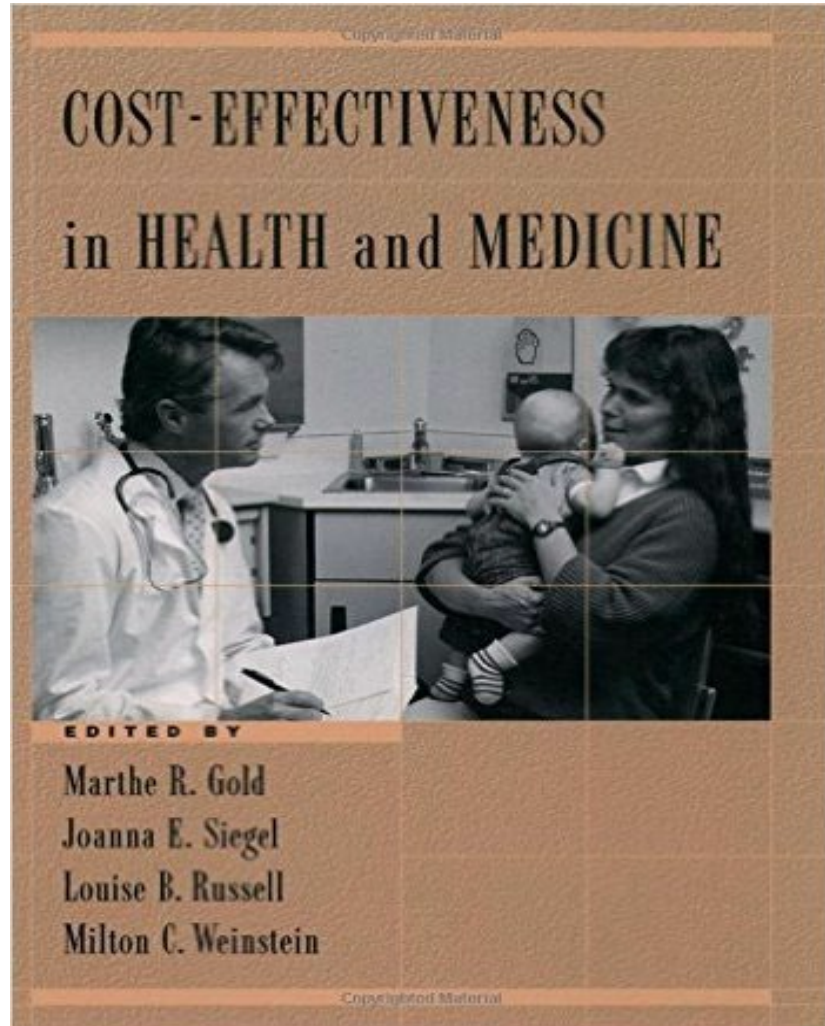


Source: Ben Stansall/Getty Images, Hannah Mckay/Reuters

# Types of analytic perspective



# Original Panel (Gold et al., 1996)



- **Reference case analysis**
  - Emphasis on QALYs
  - A societal perspective

# Societal perspective by the Original Panel

**“Who is affected? On whose behalf are decisions made?”**

**“*the societal perspective considers everyone* affected by the intervention and counts all significant health outcomes and costs that flow from it, *regardless of who experiences the outcomes or costs*”**

**“the societal perspective is the appropriate one for decision making concerning health care resources *in the public interest*”.**

Cost-Effectiveness in Health and Medicine (1st Ed), 1996, p6 and p99

# Practices in CEA since the Original Panel


Pharmacoeconomics

<https://doi.org/10.1007/s40273-020-00942-2>

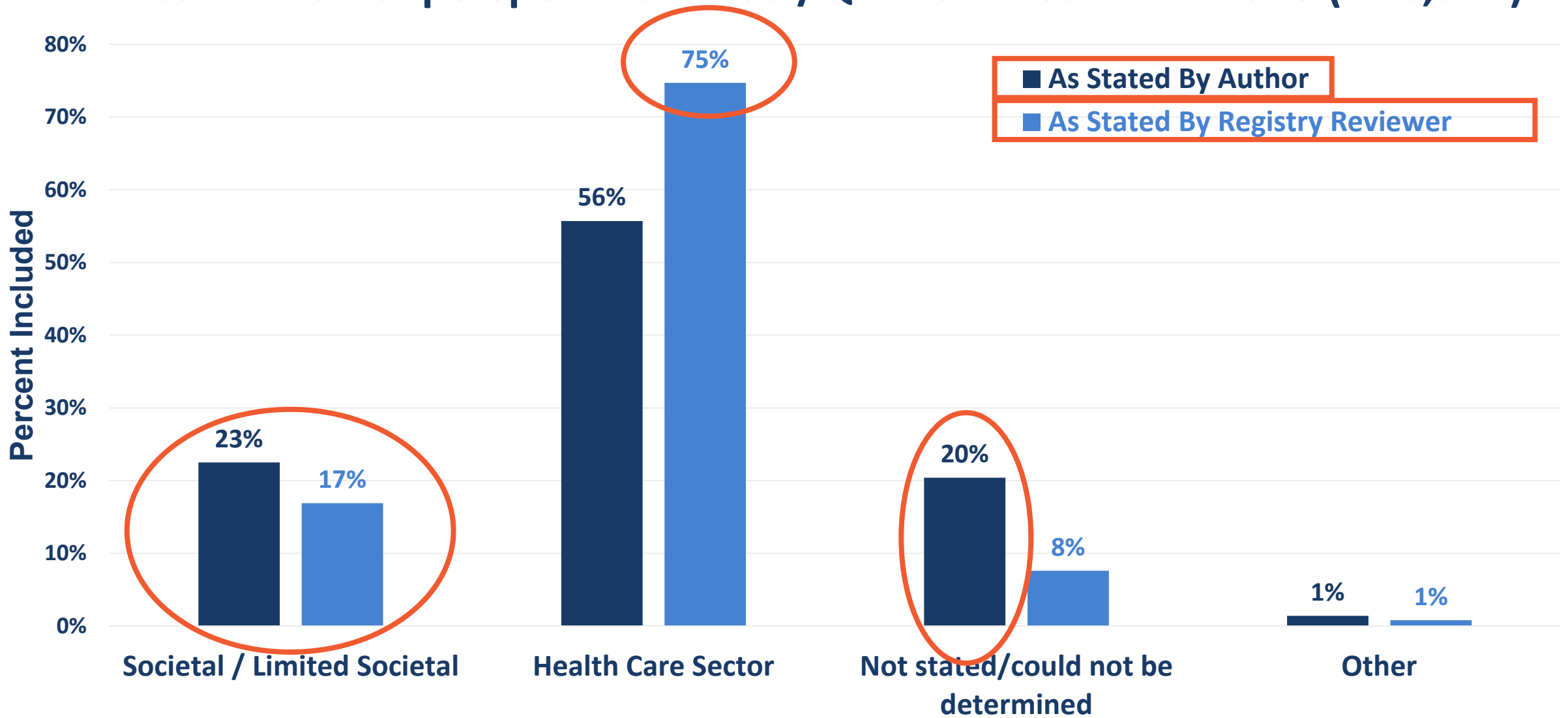
ORIGINAL RESEARCH ARTICLE



## Perspective and Costing in Cost-Effectiveness Analysis, 1974–2018

David D. Kim<sup>1,2</sup>  · Madison C. Silver<sup>1</sup> · Natalia Kunst<sup>3,4,5</sup> · Joshua T. Cohen<sup>1,2</sup> · Daniel A. Ollendorf<sup>1,2</sup> · Peter J. Neumann<sup>1,2</sup>

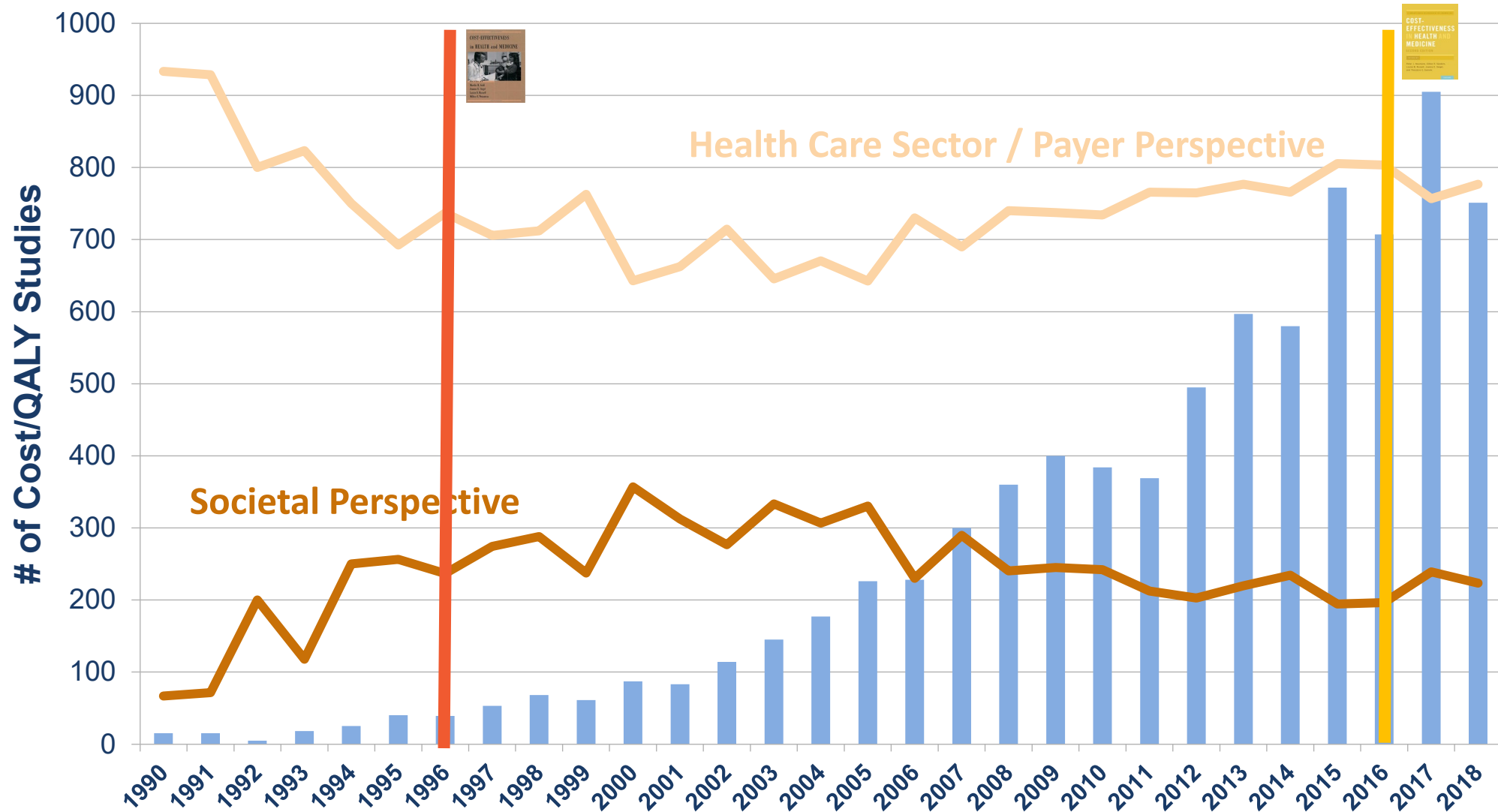
# Misclassification of perspective in cost/QALY studies: 1974-2018 (N=6,904)



Source: Kim et al. (2020, PharmacoEconomics)

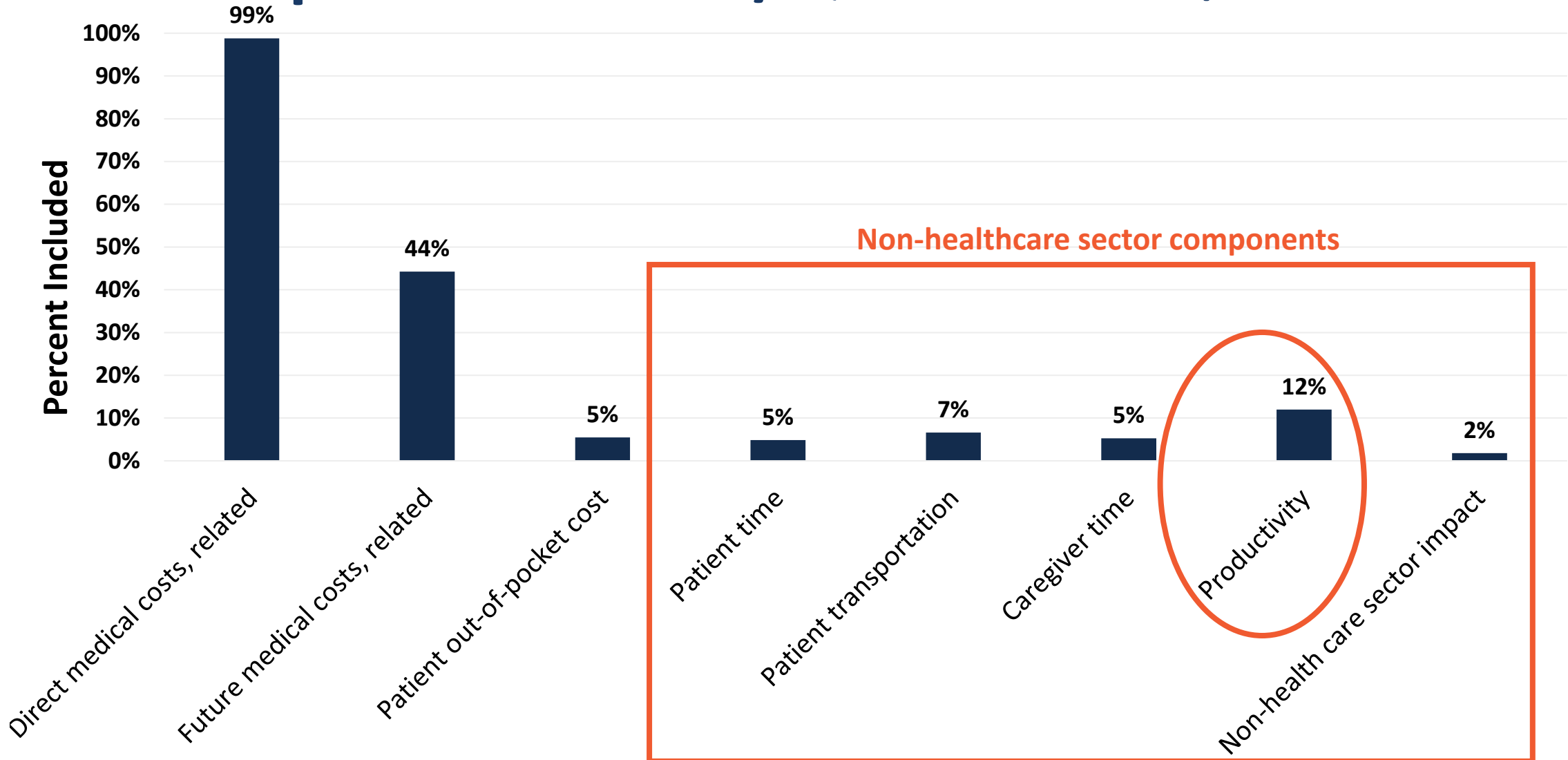


# Changes over time in analytic perspective



Source: Kim et al. (2020, PharmacoEconomics)

# Cost components in cost/QALY studies (2013-2018, N=2,839)

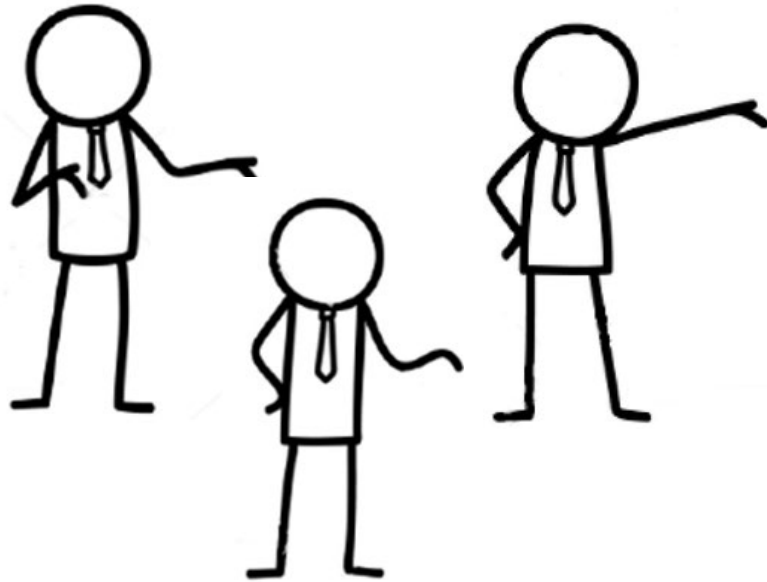


Source: Kim et al. (2020, PharmacoEconomics)

# Experiences since the Original Panel

- **Authors often misspecified or did not clearly state the perspective**
- **Many CEAs – most not using the societal perspective**
  - When applying societal perspective, important elements often omitted
- **Why?**
  - HTA guidelines often have taken more focused perspective
  - Lack of available and reliable data on non-health impact

## 2<sup>nd</sup> Panel debates: does a societal perspective make sense?



**No!**

*Whose opportunity costs?*

*No single societal perspective!*

*Revealed preference of decision-makers*



**Yes!**

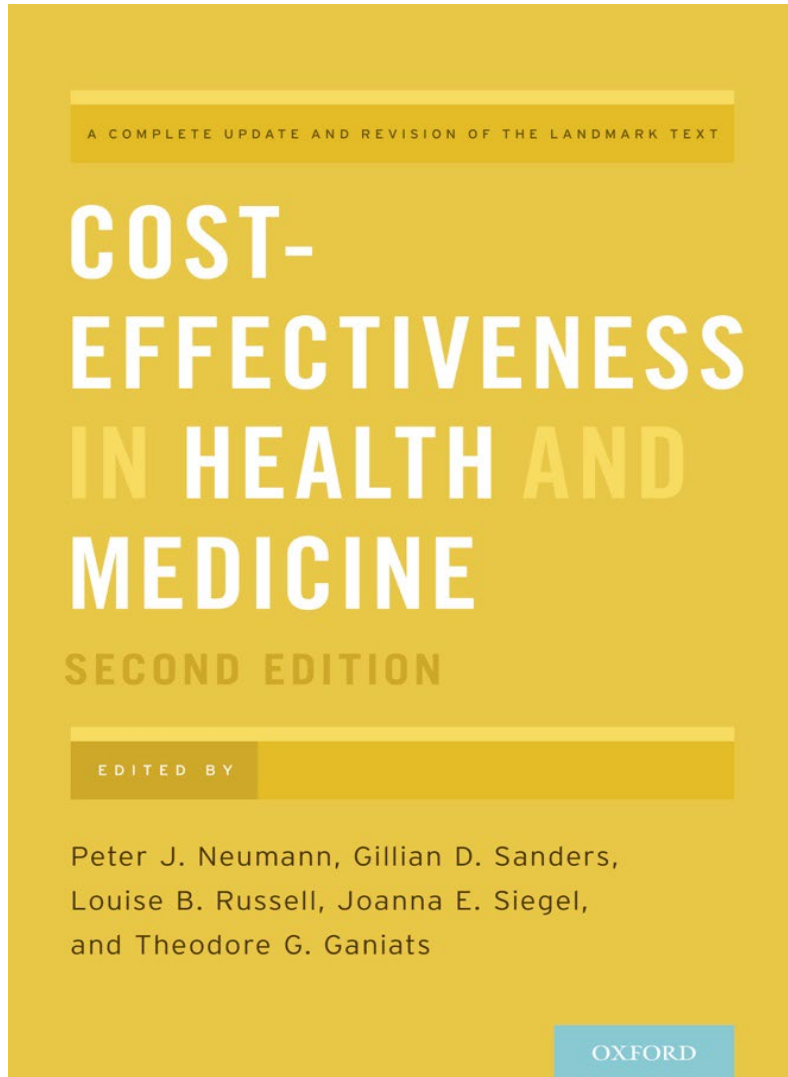
*Spillover effects*

*Elected officials (others) need to know*

*No "health budgets" in US*

*Consistency/comparability*

# Second Panel's Key Recommendations



- **Two Reference Cases (Health care & Societal)**
- **For a societal reference case:**
  - **Impact Inventory**
  - **Reporting disaggregate outcomes**

# Identifying health and non-health impact (Impact Inventory)

Sector	Type of Impact (list category within each sector with unit of measure if relevant) <sup>a</sup>	Included in This Reference Case Analysis From...Perspective?		Notes on Sources of Evidence
		Health Care Sector	Societal	
<b>Formal Health Care Sector</b>				
Health	Health outcomes (effects)			
	Longevity effects	<input type="checkbox"/>	<input type="checkbox"/>	
	Health-related quality-of-life effects	<input type="checkbox"/>	<input type="checkbox"/>	
	Other health effects (eg, adverse events and secondary transmissions of infections)	<input type="checkbox"/>	<input type="checkbox"/>	
	Medical costs			
	Paid for by third-party payers	<input type="checkbox"/>	<input type="checkbox"/>	
	Paid for by patients out-of-pocket	<input type="checkbox"/>	<input type="checkbox"/>	
Future related medical costs (payers and patients)	<input type="checkbox"/>	<input type="checkbox"/>		
Future unrelated medical costs (payers and patients)	<input type="checkbox"/>	<input type="checkbox"/>		
<b>Informal Health Care Sector</b>				
Health	Patient-time costs	NA	<input type="checkbox"/>	
	Unpaid caregiver-time costs	NA	<input type="checkbox"/>	
	Transportation costs	NA	<input type="checkbox"/>	
<b>Non-Health Care Sectors (with examples of possible items)</b>				
Productivity	Labor market earnings lost	NA	<input type="checkbox"/>	
	Cost of unpaid lost productivity due to illness	NA	<input type="checkbox"/>	
	Cost of uncompensated household production <sup>b</sup>	NA	<input type="checkbox"/>	
Consumption	Future consumption unrelated to health	NA	<input type="checkbox"/>	
Social Services	Cost of social services as part of intervention	NA	<input type="checkbox"/>	
Legal or Criminal Justice	Number of crimes related to intervention	NA	<input type="checkbox"/>	
	Cost of crimes related to intervention	NA	<input type="checkbox"/>	
Education	Impact of intervention on educational achievement of population	NA	<input type="checkbox"/>	
Housing	Cost of intervention on home improvements (eg, removing lead paint)	NA	<input type="checkbox"/>	
Environment	Production of toxic waste pollution by intervention	NA	<input type="checkbox"/>	
Other (specify)	Other impacts	NA	<input type="checkbox"/>	

- A framework for organizing, thinking about, and presenting consequences
- List of health and non-health impacts
- Ensure all consequences are considered regularly and comprehensively

# Impact Inventory: Example

## American Journal of Preventive Medicine

### RESEARCH ARTICLE

## Cost Effectiveness of Nutrition Policies on Processed Meat: Implications for Cancer Burden in the U.S.

David D. Kim, PhD,<sup>1</sup> Parke E. Wilde, PhD,<sup>2</sup> Dominique S. Michaud, ScD,<sup>3</sup> Junxiu Liu, PhD,<sup>2</sup> Lauren Lizewski, MPH,<sup>2</sup> Jennifer Onopa, MS, RDN,<sup>2</sup> Dariush Mozaffarian, MD, DrPH,<sup>2</sup> Fang Fang Zhang, MD, PhD,<sup>2</sup> John B. Wong, MD<sup>4</sup>

### INFORMAL HEALTHCARE SECTOR

### HEALTH

<input type="checkbox"/> Future related medical costs, \$	Yes	Yes	
<input type="checkbox"/> Future unrelated medical costs, \$	Yes	Yes	
<input type="checkbox"/> Patient time costs, Earnings \$	N/A	Yes	
<input type="checkbox"/> Unpaid caregiver time costs	N/A	No	No data available
<input type="checkbox"/> Transportation costs	N/A	No	No data available

# Impact Inventory: Example

## NON-HEALTHCARE SECTOR

PRODUCTIVITY	<input type="checkbox"/> Productivity (formal labor market), Earnings \$	N/A	Yes	
	<input type="checkbox"/> Uncompensated household production, patient	N/A	No	No data available
CONSUMPTION	<input type="checkbox"/> Future consumption unrelated to health, \$	N/A	No	See footnote <sup>a</sup>
SOCIAL SERVICES	<input type="checkbox"/> None	–	–	
LEGAL / CRIMINAL JUSTICE	<input type="checkbox"/> None	–	–	
EDUCATION	<input type="checkbox"/> None	–	–	
HOUSING	<input type="checkbox"/> None	–	–	
ENVIRONMENT	<input type="checkbox"/> None	–	–	



# Reporting disaggregate outcomes

- **Report intermediate health outcomes and cost categories**
- **Help compare results with other analyses that may have utilized intermediate outcomes**
- **Inform decision makers through the explicit quantification and valuation of all health and non-health impacts**

# Disaggregate outcome table: example

**Table 2.** Base-Case Results<sup>a</sup>: Lifetime Consequences for Nutrition Policies to Reduce Processed Meat Intake (All U.S. Adult Population, 250 Million)

Policy intervention	Overall health outcomes		Cancer-specific outcomes						Costs, 2014 U.S. \$, in millions				ICER, \$ per QALY	
	Life years	QALYs	CRC cases	CRC deaths	CRC PYs	SC cases	SC deaths	SC PYs	Intervention costs <sup>b</sup>	Healthcare costs	Time costs	Productivity effects	Healthcare sector perspective	Societal perspective <sup>c</sup>
Policy scenario 1: 10% excise tax														
Incremental effects vs status quo	497,000	593,000	-77,000	-55,000	-778,000	-12,500	-11,100	-57,900	1,300	-1,140	-192	-2,700	270	Cost-saving
(2.5 percentile, 97.5 percentile)	(348,000, 694,000)	(419,000, 827,000)	(-107,000, -56,800)	(-77,100, -39,500)	(-1,100,000, -533,000)	(-23,900, -6,880)	(-21,000, -5,980)	(-116,000, -26,500)	N/A	(-7,100, 1,900)	(-490, 0)	(-5,770, -1,080)		
Policy scenario 2: warning label														
Incremental effects vs status quo	553,000	660,000	-85,400	-61,300	-865,000	-15,000	-13,200	-69,400	50.3	-1,310	-213	-3,040	Cost-saving	Cost-saving
(2.5 percentile, 97.5 percentile)	(346,000, 898,000)	(418,000, 1,070,000)	(-141,000, -56,600)	(-100,000, -39,300)	(-1,440,000, -531,000)	(-34,500, -6,860)	(-30,300, -5,930)	(-167,000, -26,200)	N/A	(-8,210, 2,280)	(-613, 38.9)	(-6,930, -1,080)		

<sup>a</sup>The base-case analysis assumed a lifetime horizon and discounted future costs, life years and QALYs at 3% per year. The results reported the mean estimates with 95% uncertainty interval.

<sup>b</sup>Policy intervention costs represented the net present value over 30 years of the effective period with a 3% discount rate. The impact of nutrition policies was assumed one-time effect that would last at the reduced processed meat intake.

<sup>c</sup>A societal perspective included healthcare costs, time costs associated with receiving medical care, and productivity effects.

CRC, colorectal cancer; ICER, incremental cost-effectiveness ratio; LY, life year; PY, person-year; QALY, quality-adjusted life year; SC, stomach cancer.

Source: Kim et al. (2019, Am J Prev Med)

# Implications

- **Perspectives matter in value assessment!**
  - Decisions based on incomplete valuation may not optimize overall welfare
- **Importance of transparent reporting**
  - Impact Inventory and reporting disaggregated outcomes can help
- **Challenges remain in the lack of available data**
  - Valuing non-health outcomes
  - Valuing effects of others

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# Getting to value: measurement questions

- Impacts beyond health?
- What role for QALYs?
- Drug launch price or price over time?
- How to address uncertainty?

# Getting to value: policy questions

- On what basis does Medicare *negotiate*?
- What role of private payers?
- Does the US need a new public HTA Institute?

# And a word of caution

- By themselves, value-based prices will not make drugs affordable
- They do help balance innovation and other priorities
- For affordability – other reforms needed

# Conclusion

“ Paying value-based prices, even as we strive to encourage innovation, makes sense because it helps ensure that drug companies produce what people want—products that improve people’s health—while considering society’s other pressing priorities.”

- The Right Price



# Thank you!

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