

The Next Generation of Value Assessment: *Including the Patient Voice*

November 12, 2019

Washington, D.C.- Ronald Reagan Building



The Next Generation of Value Assessment: *Including the Patient Voice*

November 12, 2019

Washington, D.C.- Ronald Reagan Building

Session Purpose

To inform stakeholders about useful tools and findings PhRMA Foundation grant recipients are developing to overcome shortcomings of current approaches to value assessment. The conference will also illustrate the connection between value assessment research and the practical applications to support and strengthen the decision-making process within the U.S. healthcare system



Welcome

Eileen Cannon
President

PhRMA Foundation

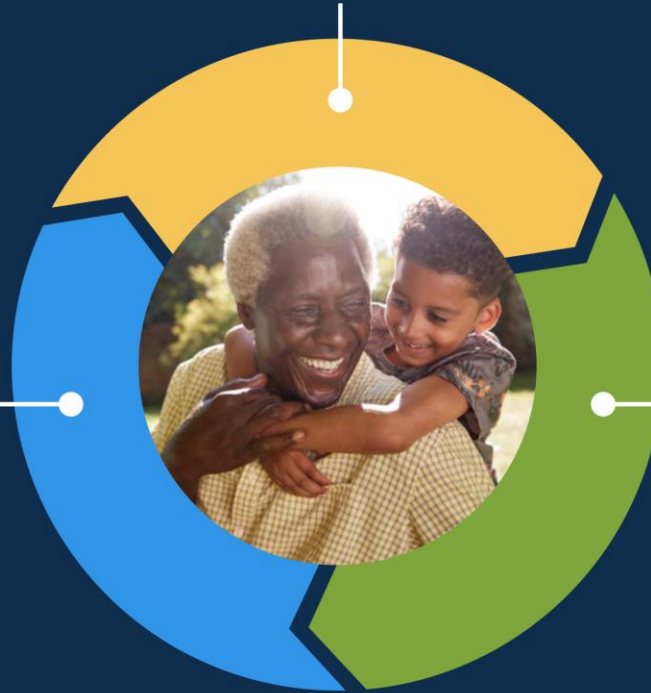




Improving public health by proactively investing in innovative research, education and value-driven health care

VALUE ASSESSMENT INITIATIVE

**YOUNG SCIENTIST
FELLOWSHIP AND
GRANT PROGRAM**



**DATA AND
TECHNOLOGY
PROGRAM**

PROVIDING **OVER \$3.6 MILLION ANNUALLY** IN PEER-REVIEWED AWARDS

The PhRMA Foundation funds collaborative efforts that promote innovative research, support emerging data science and drug discovery, and advance better methods to accurately characterize the value of outcomes for a wide variety of stakeholders.

PhRMASFoundation.org

Why Value Assessment is Important from a Patient's Perspective

Jaime M. Sanders

Migraine Patient Advocacy Coordinator



The Next Generation of Value Assessment: *Including the Patient Voice*

November 12, 2019

Washington, D.C.- Ronald Reagan Building

Panel #1: PhRMA Foundation Grant Recipients
Highlight New Approaches to Value Assessment

Working to transform value assessment to ensure it is patient centered, appropriately capturing the value of innovation and useful to decision-makers

Moderator:

Sachin Kamal-Bahl, PhD (COVIA Health Solutions)

Panelist:

Susan dosReis, PhD (PAVE)

George Miller, PhD (RC-HCVA)

Jon Campbell, PhD (pValue)

Peter Neumann, ScD (CEVA)



Moderator



Sachin Kamal-Bahl, PhD
President and Founder
COVIA Health Solutions

Value Assessment Advisory Committee Member
PhRMA Foundation

PhRMA Foundation Value Assessment Initiative

The PhRMA Foundation created the Value Assessment Initiative to address challenges in assessing the value of medicines and health care services by supporting the development of robust, patient-centered methodologies.



Value Assessment Landscape

- Concern over rising U.S. health care costs in recent years has increased interest in promoting high-quality care, while avoiding low value or inefficient care
- In response, a number of initiatives aiming to drive value in health care have emerged, but few offer transformative solutions that reflect patient preferences and real-world clinical practice
- In addition, many issues in methodology and patient engagement remain unresolved



The PhRMA Foundation Value Assessment Initiative seeks to support activities that lead to the development and application of high-quality, patient-centered approaches to value assessment

Value Assessment Initiative: Program Goals

The primary goals of the Value Assessment Initiative are to develop tools to advance value-based healthcare and patient-focused solutions, and build partnerships with key organizations and stakeholders.

Program Goals

- 1** The ideal Program for the value assessment initiative will develop tools to advance value-based healthcare, patient-focused solutions, and build partnerships with key organizations and stakeholders

- 2** Create a Program with cross-cutting value across the PhRMA membership to advance patient-focused solutions for emerging challenges

- 3** Opportunity to build strong partnerships with influential organizations and stakeholders

Value Assessment Initiative: Funding Criteria and Framework

The Initiative aims to support the development of methods to assist healthcare stakeholders in making informed decisions to improve healthcare efficiency through challenge, research, and centers of excellence awards.

Funding Criteria

- Assist stakeholders, including patients, providers and payers, in making informed decisions to improve health and care efficiency
- To maximize impact, these methods must offer opportunities to incorporate patient characteristics and their preferences to guide treatment decisions

Award Framework

Challenge Awards

What are innovative, patient-centered approaches to contribute to healthcare value assessment that move beyond the inherent limitations of analyses based on the quality-adjusted life year metric?

\$85K Granted Across 3 Challenge Awards

Research Awards

How can we address limitations with available data sources, methods, and measures to integrate patient perspectives into value assessment?

\$300K Granted Across 3 Research Awards

Centers of Excellence

Establish and sustain new collaborative, multi-disciplinary centers that will undertake activities to build evidence and partnerships that can inform value assessment strategies and value-driven decision-making.

\$2MM Granted Across 4 Center Awards

Value Assessment Centers of Excellence

Center for Patient- Driven Value Assessment (PAVE)



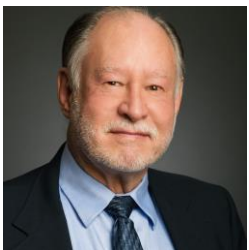
Susan dosReis, PhD, FISPE
University of Maryland

Center for Pharmaceutical Value (pValue)



Jonathan D. Campbell, PhD
University of Colorado

Research Consortium for Healthcare Assessment (RC-HCVA)



George Miller, PhD
Altarum and VBID Health

Center for Enhanced Value Assessment (CEVA)



Peter J. Neumann, ScD
Tufts Medical Center



Center for
*P*atient-Driven
*V*alues in Healthcare
*E*valuation



PAVE

PATIENT-DRIVEN
VALUES *in* HEALTHCARE
EVALUATION

- PAVE Center – who we are and our mission
- Contribution to value assessment
- Partnerships
- Work in progress towards our goal
- Forthcoming activities
- Accomplishments

University of Maryland School of Pharmacy

- *Wendy Camelo Castillo*
- *Susan dosReis*
- *Joey Mattingly*
- *Daniel Mullins*
- *Julia Slejko*

National Health Council

- *Marc Boutin*
- *Eleanor Perfetto*
- *Elisabeth Oehrlein*

In Partnership With
Patient Community Leaders, Payer &
Industry Stakeholders

Funded by
Pharmaceutical Research and
Manufacturers of America (PhRMA)
Foundation

- Engage patient and other stakeholder partners in setting our operational and research agenda.
- Provide training in value assessment for minority and underserved patient communities.
- Incorporate patient-informed value elements into economic evaluations.
- Disseminate findings to patient and research communities.

PATIENT-DRIVEN VALUE ASSESSMENT

PAVE's fundamental premise is that value in health care must be defined by patients and value assessment must reflect heterogeneity across patients. Our multi-stakeholder collaborations aid us in generating value tools that create opportunities to support patient-centered health care decision-making and support other stakeholders in their engagement of patients.

TRADITIONAL PATH

The traditional path is informed by randomized trial and research data without patient input.

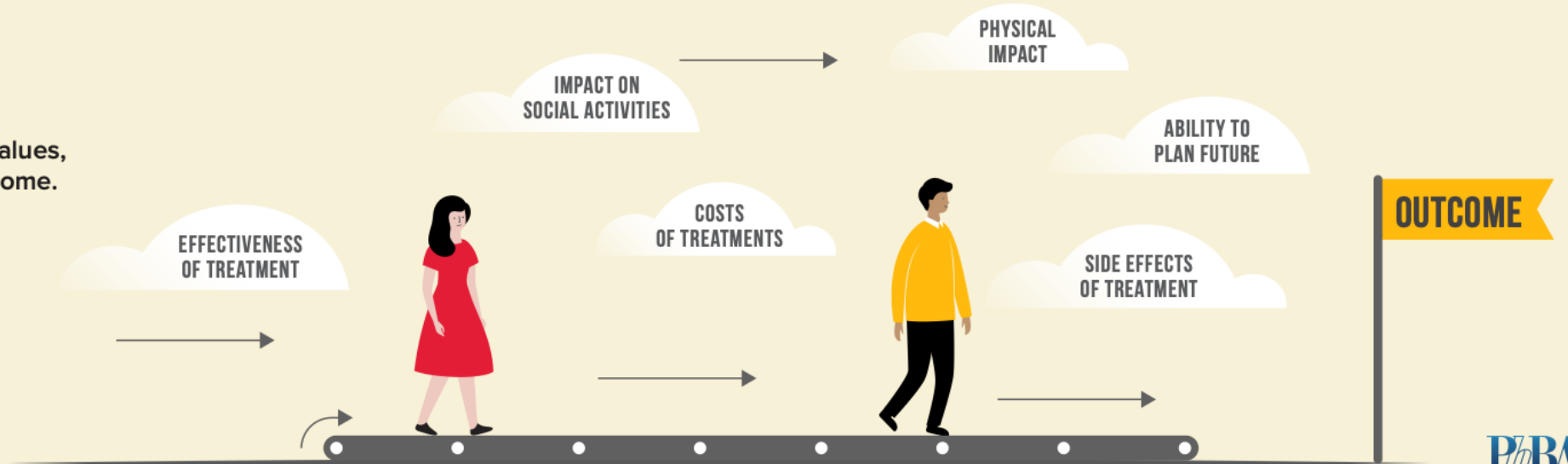


OUTCOME

A PATIENT'S JOURNEY TOWARDS BETTER OUTCOME

✓ PAVE PATH

The PAVE path is informed by patient values, guiding the patient towards better outcome.



- Create a nuanced understanding of patient values in healthcare evaluation
 - Identify novel value elements that are informed by patient experiences
 - Test different approaches in using patient-informed value elements
- Incorporate this information into an economic evaluation
- Establish a set of resources to benefit the field

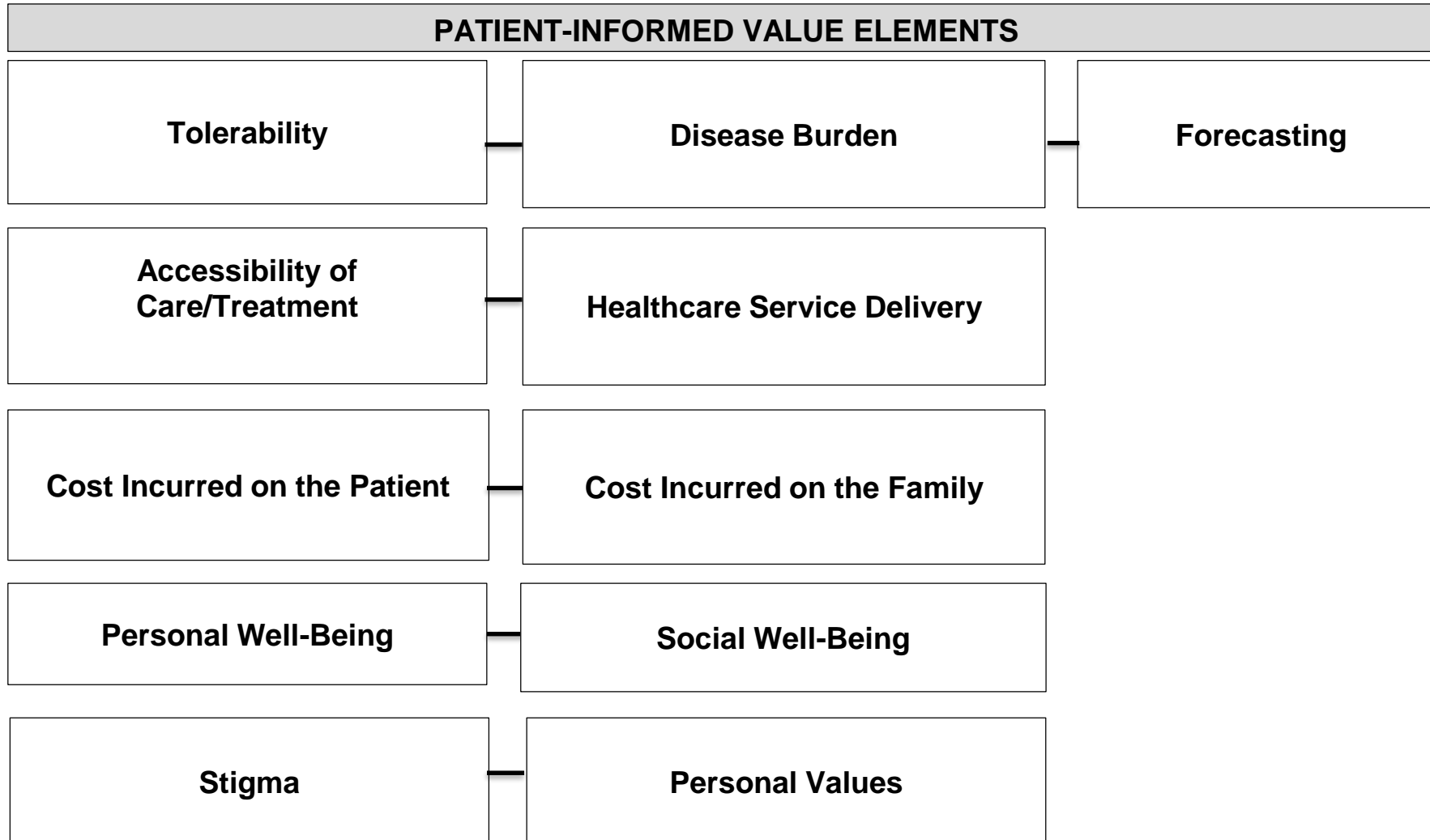
- Ongoing partnerships with patient communities
 - Chronic Obstructive Pulmonary Disease (COPD) Foundation
- Patient stakeholder representation on the advisory committee
- Anticipated partnerships
 - Center for Medical Technology Policy (CMTP)
 - Innovation and Value Initiative (IVI)

Who we engaged....

- Patient stakeholders from our Advisory Committee
 - One member represented the Hispanic community
- National Health Council (NHC) Value Workgroup Members (14 diverse patient communities)

What we did....

- Elicited and prioritized value elements that are important to patients:
 - **Phase 1:** *Develop a list of existing value elements from the literature*
 - **Phase 2:** *Elicit elements of value from patient stakeholders on our advisory committee*
 - **Phase 3:** *Prioritize and refine the value elements with a range of patient communities*



- Research Core & the COPD Value Elements
 - Evaluate new model inputs
 - Adjust existing health-state utilities
 - Examine value for subgroups based on heterogeneity of preferences
- Education Core Webinars
 - Patient Involvement in Value Assessment: Insights from Abroad
 - Introduction to Multi-Criteria Decision Analysis
 - Value Assessment in Medicaid
- Dissemination Core
 - Patient-Informed Value Elements Conceptual Framework
 - Relating Value Elements to Previous COPD CEA/Economic Evaluations

- Education Materials:
 - Modules for sensitivity analysis and heterogeneity of treatment effects
 - Guide to help patient-group provide comments on a value assessment
 - Conducted two trainings for patient communities
 - One breakout session resulted in a guide entitled “What I Wish I Had Known”
- Research Materials:
 - Mapping existing patient preference research to value elements
 - 5 different medical conditions
 - Developed methodological approach to apply patient-informed value elements to a specific patient community/condition
- Dissemination Materials:
 - PAVE webpage ([PAVE Center](#))
 - 3 publications, 1 in review, and 2 manuscripts in progress
 - Partnership to Improve Patient Care panel
 - Alliance for Health Policy Summit panel



Thank You

RESEARCH CONSORTIUM FOR HEALTH CARE VALUE ASSESSMENT (RC-HCVA)

George Miller, Altarum Center for Value in Health Care
November 12, 2019



Our Mission



RC-HCVA is a joint initiative of Altarum and VBID Health whose mission is to promote the pursuit of value in health care delivery in the U.S. by identifying high-and low-value clinical services, tracking the use of such services, and helping to ensure that consumer preferences are incorporated in health care decisions.



RESEARCH CONSORTIUM
for Health Care Value Assessment

How We Accomplish Our Mission

- Conduct research
 - Methods
 - Measurement
- Develop research briefs and concept papers
 - Document research results
 - Address related issues of value
- Collaborate
 - Altarum/VBID Health partnership
 - Advisory group
 - 350 “Colleagues in Value”
- Disseminate
 - Consortium web site
 - Quarterly newsletter
 - Presentations, blogs, publications

How We Contribute to Value Assessment: Measuring Low-Value Care

- Current methods analyze claims data
- Approach incorporates time series measurements to track progress
- Results are extrapolated to national level
- Working toward comprehensive measurement (See Miller et al., “A Framework for Measuring Low-Value Care”, *Value in Health*, 2018)

How We Contribute to Value Assessment: Extensions Beyond Low-Value Care Measurement

- Helping focus targeted interventions to reduce low-value care
- Incorporating measurement of high-value care
- Developing a standardized waste reporting tool
- Investigating potential for a screening tool to identify low-value care risks in a population

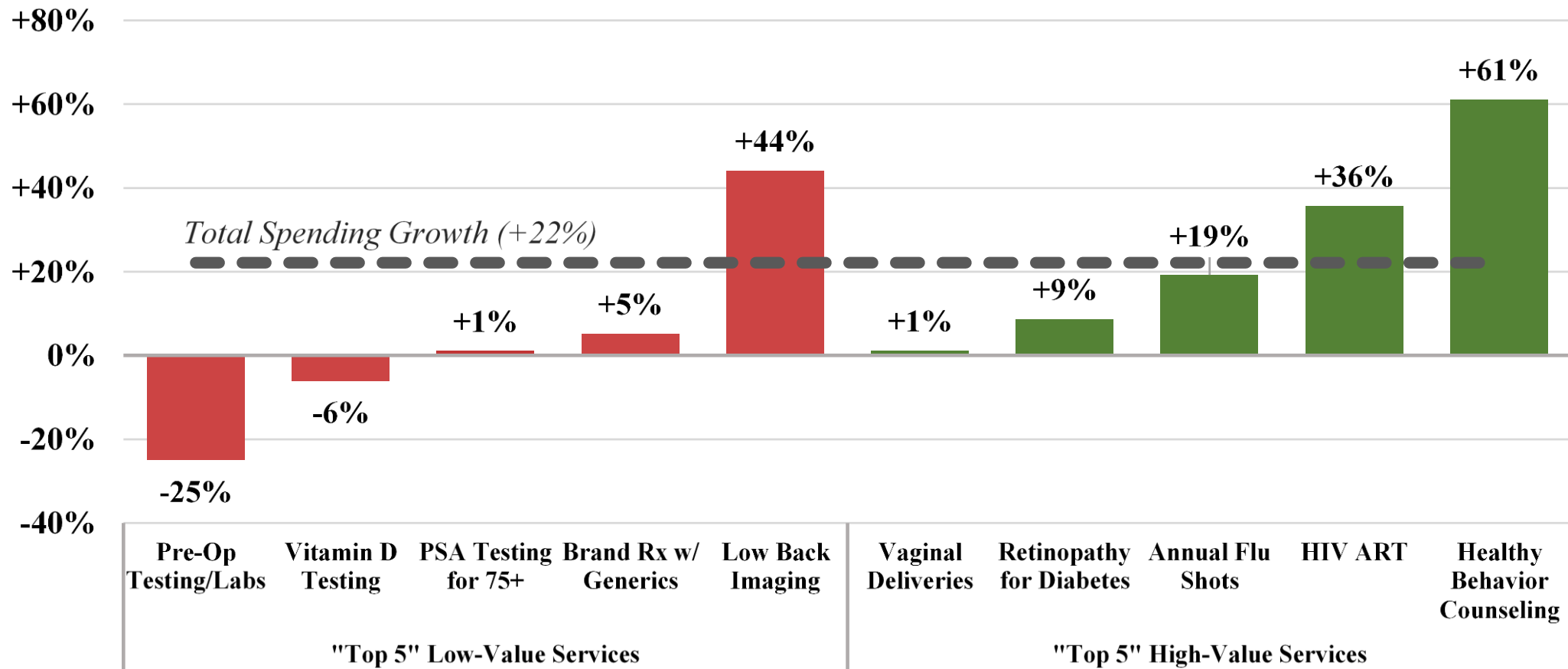
Our Team

- Altarum
 - George Miller, Co-Director
 - Beth Beaudin-Seiler, Manager
 - Other Altarum staff as needed
- VBID Health
 - Mark Fendrick, Co-Director
 - Michael Budros
- Advisory Group
 - David Meltzer, University of Chicago (Chair)
 - Beth Bortz, Virginia Center for Health Innovation
 - Peter J. Neumann, Tufts Medical Center
 - Neel Shah, Harvard Medical School
 - Steven M. Teutsch, UCLA and USC
- Other Collaborators as Needed

Previous and Forthcoming Research Output

- Concept Paper No. 1: *Improving Health by Reducing Low-Value Care*
- Concept Paper No. 2: *A Framework for Addressing Low-Value Care*
- Concept Paper No. 3: *Efforts to Measure Value in Health Care: Greater Balance is Needed*
- Concept Paper No. 4: *An Employer-Based Health Care Waste Indicator Tool: Prospects, Potential and Problems*
- Research Brief No. 1: *The "Top 5" Low- and High-Value Services: Trends in Health Care Spending Among the Privately Insured, 2014-2016* (May 2019)
- Forthcoming: Research Brief No. 2 will develop national and state-level estimates of low-value spending on 20 services

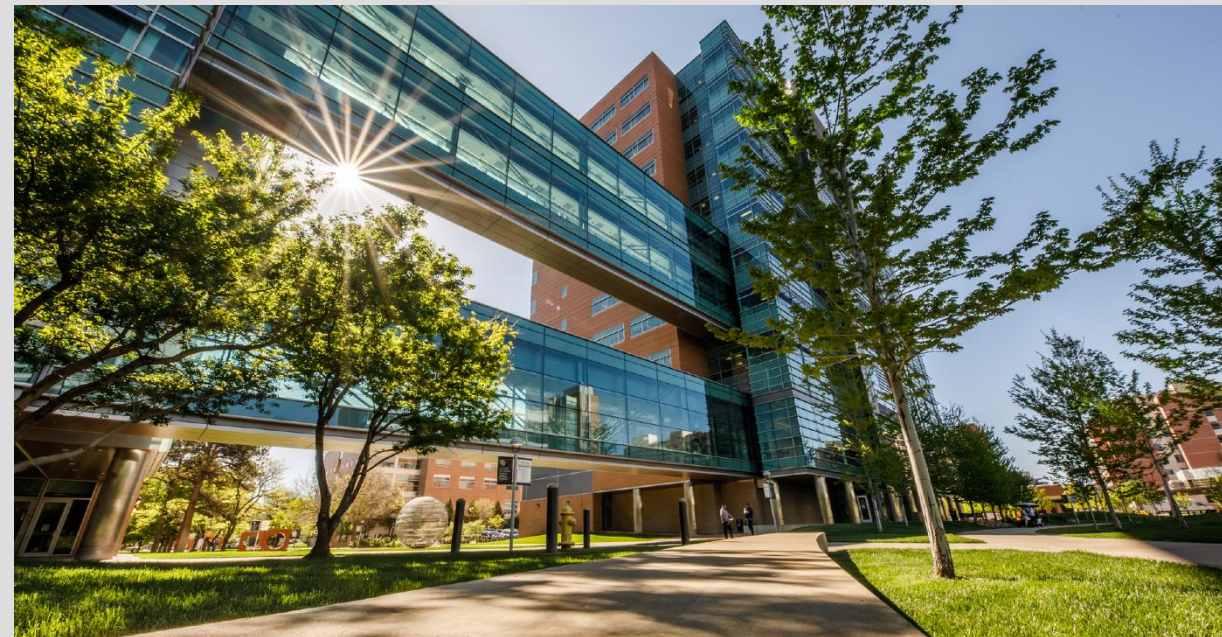
Research Results From QS 1: Spending Growth for Selected Services (U.S. Privately-Insured Population, 2014Q1 - 2016Q4)



Pharmaceutical Value (pValue) University of Colorado

The Next Generation of Value Assessment

November 12, 2019



pValue Mission and Vision

- Mission: Apply and test novel US healthcare value assessment methods to guide population-level decision making.
- Vision: Leader in conducting and advancing the science of US healthcare value assessment.
- Guiding principles:
 - Science leads
 - Value is heterogeneous
 - Useful evidence yields improved decisions

Limitations of Traditional Value Assessment

- Evidence from cost-effectiveness analysis (CEA)
 - CEA is a starting point for traditional value assessment
 - Threshold links cost-effectiveness findings to value interpretation
- CEA includes standardized methods conditioned on assumptions and inputs
 - By definition, does not account for non-traditional “it depends” value criteria
 - By definition, is not fully comprehensive

US Value Interpretations... *It Depends*

- Criteria influencing US value interpretations and corresponding stated preference votes include:
 - disease state (e.g. cancer or ultra-rare diseases)
 - caregiver burden
 - productivity
 - disease severity
 - lack of evidence
 - uncertain benefits compared to alternatives
 - safety concerns

Potential of Novel Value Assessment Methods

- Value assessment characterized by multiple, sometimes conflicting criteria (*“it depends”*)
- Multi-Criteria Decision Analysis (MCDA): useful technique to enable more structured and objective decision-making
 - Value main subdomains: costs and outcomes
 - MCDA is most useful in outcomes domain
 - Qualitative MCDA
 - Decision based on deliberations of explicitly defined criteria (criterion measurement specified, but weights not specified)
 - Quantitative MCDA
 - Produces a score used as a decision aid (criterion measurement specified and weights specified)

pValue Objectives

- Review applications of MCDA and where it may show promise for use in US value assessment
- Educate stakeholder communities on MCDA techniques
- Develop pilot MCDA tools for innovative therapies (e.g. cancer or ultra-rare diseases)
- Partner with patient, payer, and other stakeholder groups to identify and compare criteria of value that are important to them
- Test impact of adding MCDA to traditional value assessments, versus traditional value assessment alone, on health care decision making

A CLEAR PATH

TOWARD ACHIEVING VALUE IN HEALTH

The U.S. health system can improve value in health through the understanding and alignment of all stakeholders. **Multi-criteria decision analysis (MCDA)** can aid evidence generation through identifying important criteria of value outside the traditional understanding.



PAYER

PROVIDER



WHAT IS A FAIR PRICE?

HOW IMPORTANT IS AFFORDABILITY?

SHOULD VALUE DEPEND ON SEVERITY OF DISEASE?

NO OUTLET

MCDA BRINGS STRUCTURE AND A VOICE TO ALL STAKEHOLDERS.

VALUE-BASED HEALTH SYSTEM

Studies led by pValue will suggest where MCDA can aid value-based decisions.

HOW IMPORTANT IS THE QUALITY OF EVIDENCE?

HOW DO YOU INCLUDE CAREGIVER BURDEN IN VALUE?

WHAT ROLE DOES NOVELTY PLAY IN VALUE?

PLAN MEMBER

DEAD END



DETOUR

RESEARCHER



INNOVATOR

pValue Active Efforts

- MCDA white paper “Complementing Coverage and Reimbursement Decisions With Multi-Criteria Decision Analysis,” available on [American Journal of Managed Care Contributor Page](#)
- Organization and integration of research steering committee
- Systematic literature review (in collaboration with Syreon Research Institute)
- Focus group with oncology patients to identify value criteria (in collaboration with Cancer Support Community)
- Engagement with payers to identify value criteria (in collaboration with Real Endpoints)

pValue MCDA Applications for Year 2020

- Develop qualitative MCDA tools that identify novel outcomes criteria
 - By stakeholder and application (e.g. ultra rare disease and oncology)
 - Compare and contrast outcomes criteria important to patients, payers, and other stakeholders
- Develop quantitative MCDA as decision tools (not rules)
 - Focus on outcomes criteria outside traditional value (outside of cost and QALYs)
 - Pilot test applications that include traditional value assessment and novel value assessment tools

University of Colorado pValue Investigators

Jon.Campbell@cuanschutz.edu

Robert.McQueen@cuanschutz.edu

Melanie.Whittington@cuanschutz.edu

THE CENTER FOR ENHANCED VALUE ASSESSMENT (CEVA)

November 12, 2019

Peter J. Neumann, Sc.D.

Center for the Evaluation of Value and Risk in Health (CEVR)

Tufts Medical Center, Boston

“CEVA” is how you pronounce “CEVR” in Boston!



CEVA's Mission

- Explore the incorporation of additional elements into traditional cost-effectiveness analyses

Motivation #1

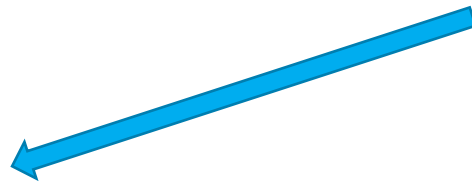


2nd Panel recommends cost/QALYs



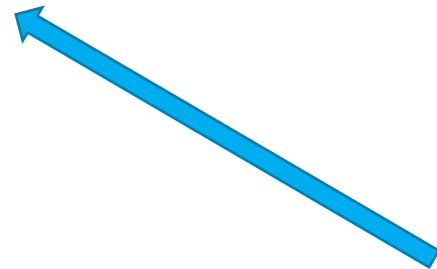
Cost

(Costs with treatment)
– (Costs without treatment)



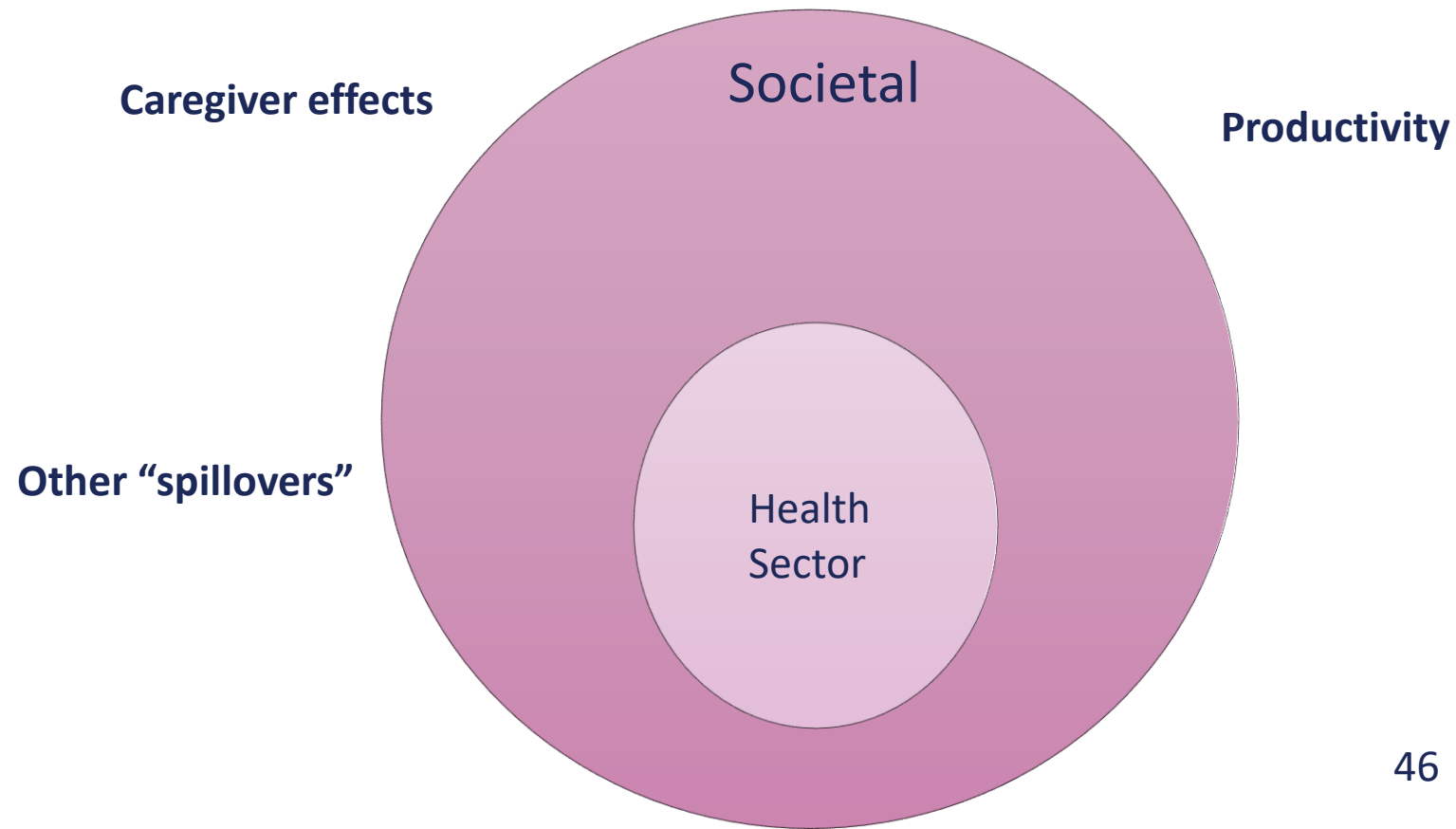
QALYs

(QALYs with treatment)
– (QALYs without treatment)

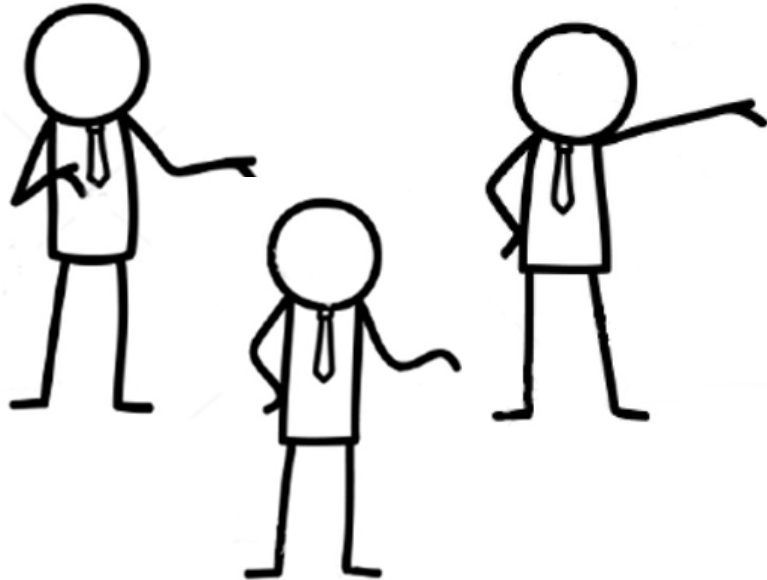


But what elements to include...?

- That depends (in part) on perspective



The 2nd Panel debates... does a societal perspective make sense?



No!

Whose opportunity costs?

No single societal perspective!



Yes!

Broad impacts/Spillovers!

The public interest!

Consistency/comparability

The Second Panel's solution...

- Do it both ways...conduct both a health care and societal perspective
- And include an “Impact Inventory”

Sector	Type of Impact (list category within each sector with unit of measure if relevant) ^a	Included in This Reference Case Analysis From...Perspective?		Notes on Sources of Evidence
		Health Care Sector	Societal	
Formal Health Care Sector				
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"/>	
Health	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"/>	
Informal Health Care Sector				
Health	Patient-time costs	NA	<input type="checkbox"/>	
	Unpaid caregiver-time costs	NA	<input type="checkbox"/>	
	Transportation costs	NA	<input type="checkbox"/>	
Non-Health Care Sectors (with examples of possible items)				
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 ^b	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"/>	

Motivation #2

VALUE IN HEALTH 21 (2018) 161–165



ELSEVIER

Available online at www.sciencedirect.com

ScienceDirect

journal homepage: www.elsevier.com/locate/jval



A Health Economics Approach to US Value Assessment Frameworks—Summary and Recommendations of the ISPOR Special Task Force Report [7]

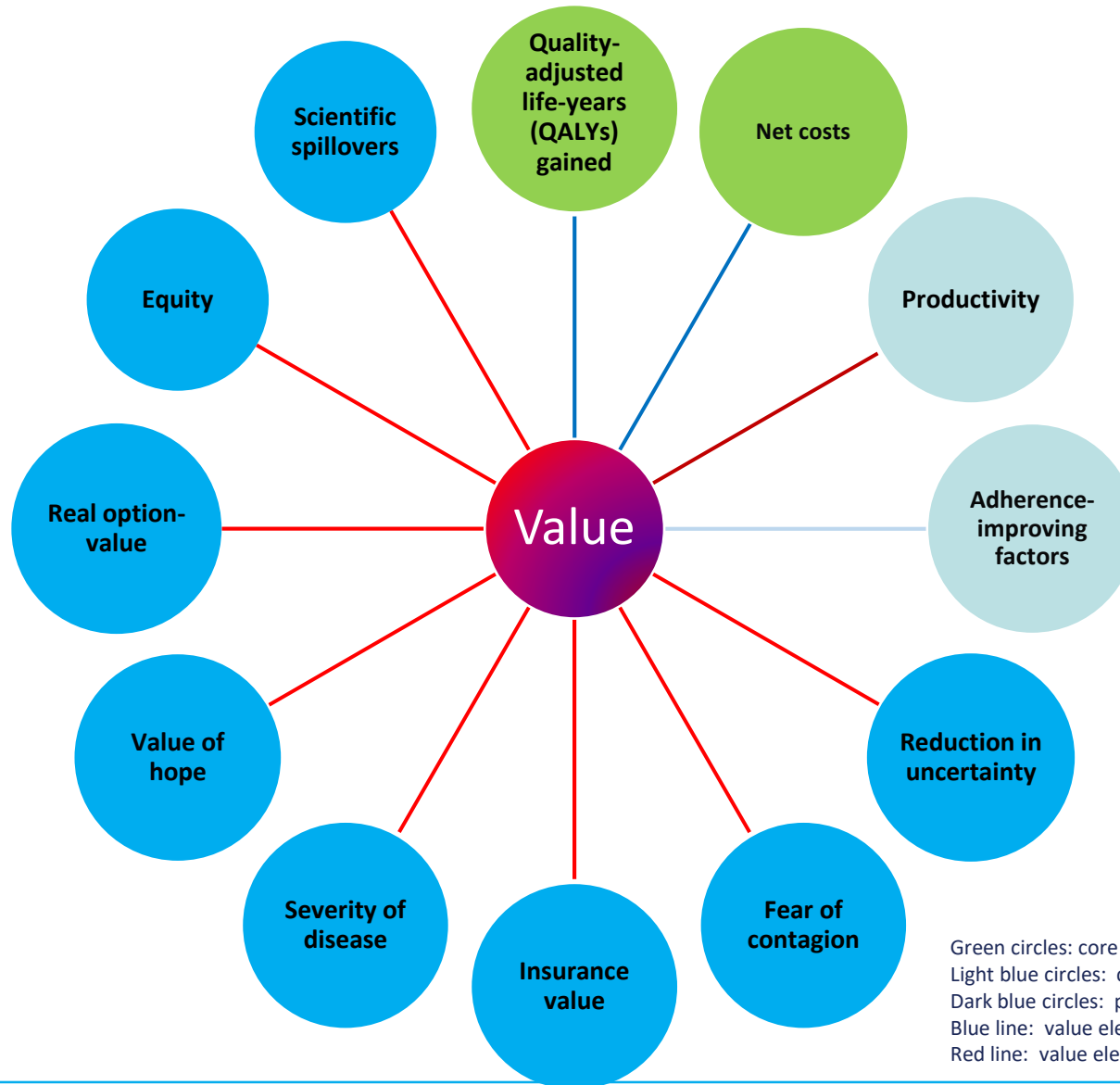


Louis P. Garrison Jr, PhD^{1,*}, Peter J. Neumann, ScD², Richard J. Willke, PhD³, Anirban Basu, PhD¹, Patricia M. Danzon, PhD⁴, Jalpa A. Doshi, PhD⁵, Michael F. Drummond, PhD⁶, Darius N. Lakdawalla, PhD⁷, Mark V. Pauly, PhD⁴, Charles E. Phelps, PhD⁸, Scott D. Ramsey, MD, PhD⁹, Adrian Towse, MPhil, MA¹⁰, Milton C. Weinstein, PhD¹¹

¹Pharmaceutical Outcomes Research and Policy Program, The Comparative Health Economics Institute, University of Washington, Seattle, WA, USA; ²Center for the Evaluation of Health Care, Boston, MA, USA; ³International Society for Pharmacoeconomics and Outcomes Research, Philadelphia, PA, USA; ⁴University of Pennsylvania, Philadelphia, PA, USA; ⁵Centre for Health Economics, University of York, York, UK; ⁶Schaeffer Center for Health Policy and Economics, University of Southern California, Los Angeles, CA, USA; ⁷Economics, Public Health Sciences, Political Science, University of Rochester, Gualala, CA, USA; ⁸Department of General Internal Medicine, University of Washington, Seattle, WA, USA; ⁹Office of Health Economics, London, UK; ¹⁰Health Policy and Management, Harvard University, Boston, MA, USA

Augment the
QALY?

ABSTRACT



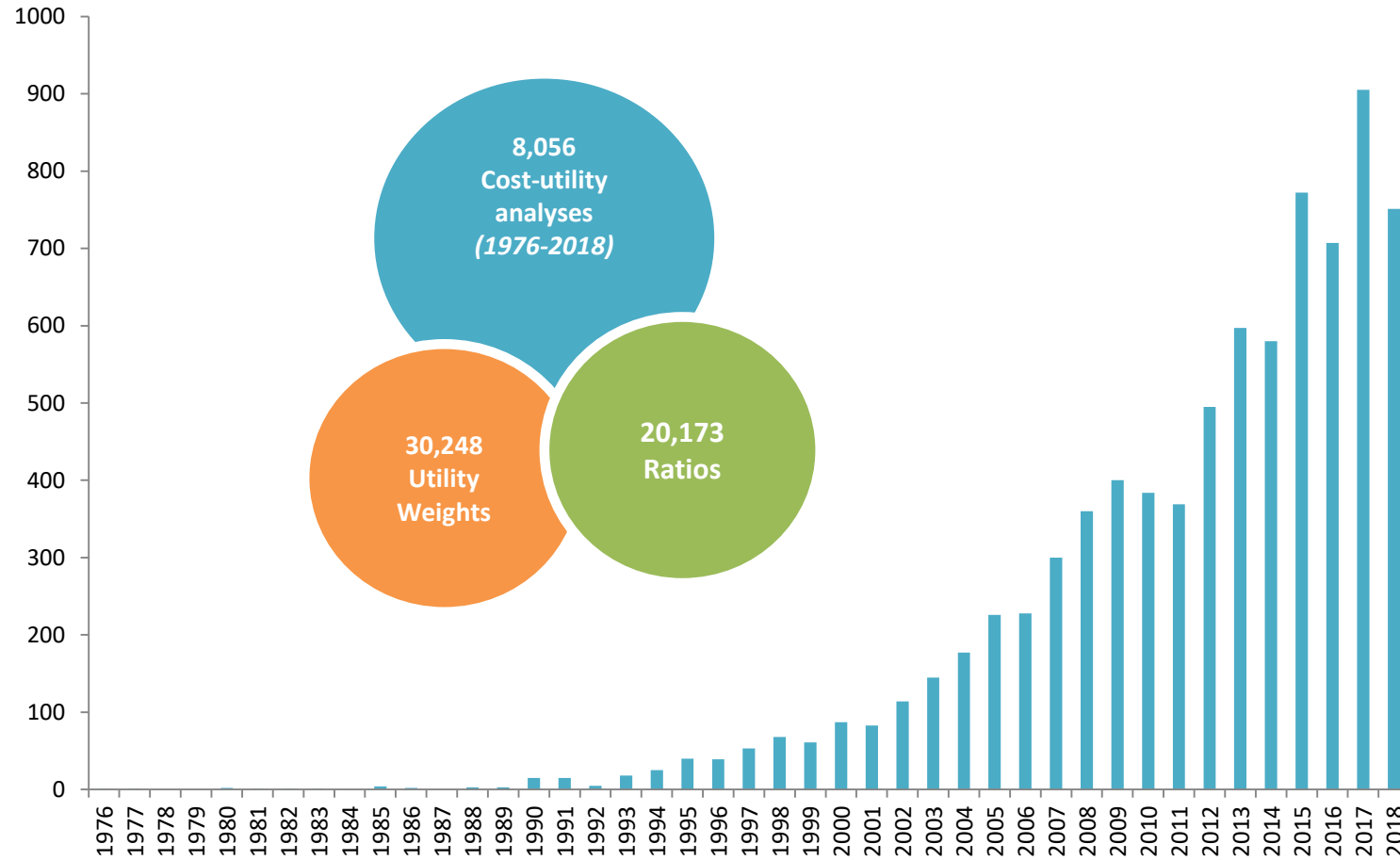
Green circles: core elements of value
 Light blue circles: common but inconsistently used elements of value
 Dark blue circles: potential novel elements of value
 Blue line: value element in traditional payer perspective
 Red line: value element also included in societal perspective

CEVA activities

- Explore whether published CEAs include broader value elements
- Conduct CEA case studies to incorporate these elements
- Characterize patient views on these elements
- Explore a user-friendly dashboard

New CEVA analyses!

of Published Cost/QALY Articles

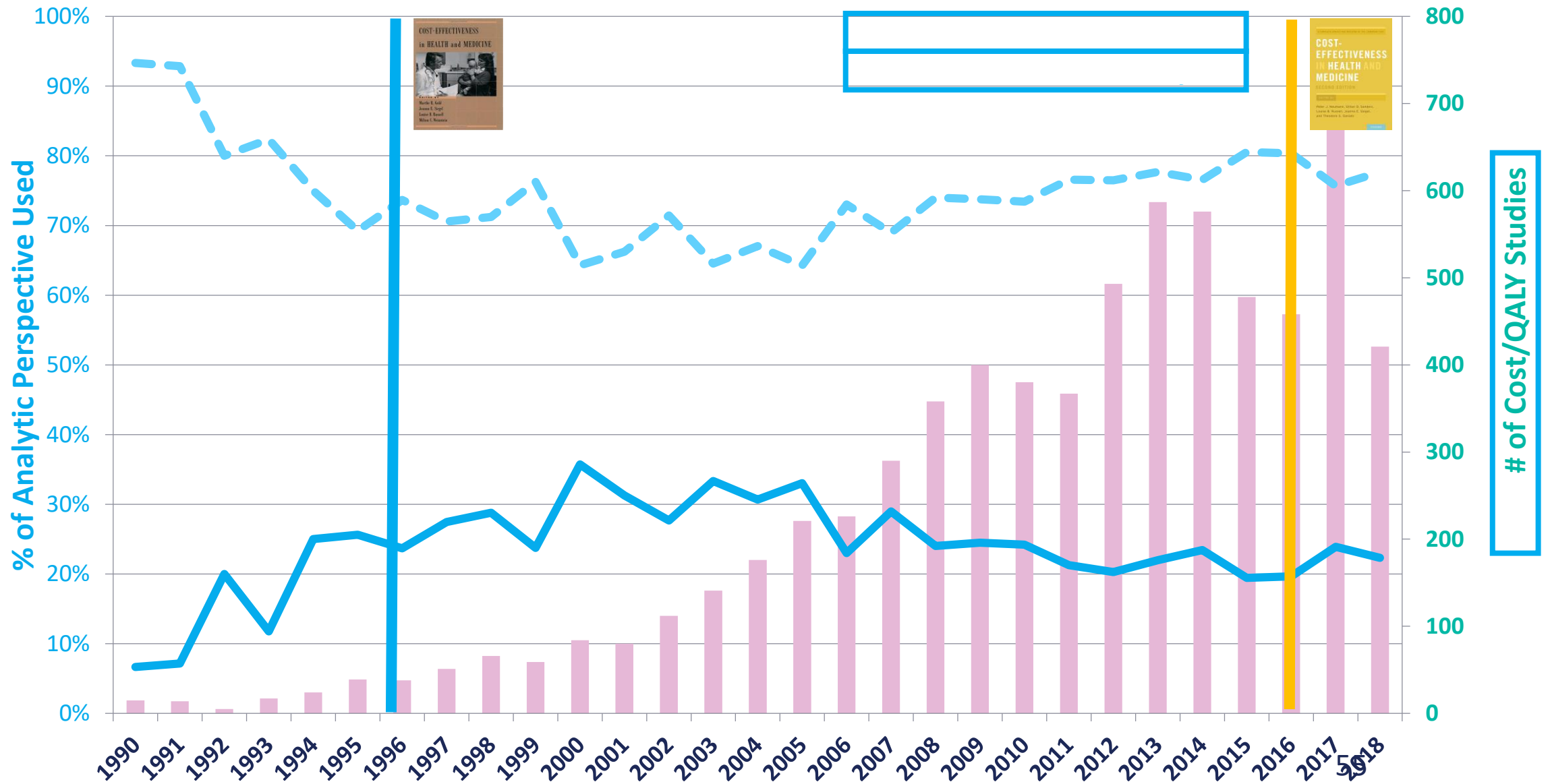


Source: Tufts MC CEA Registry, www.cearegistry.org

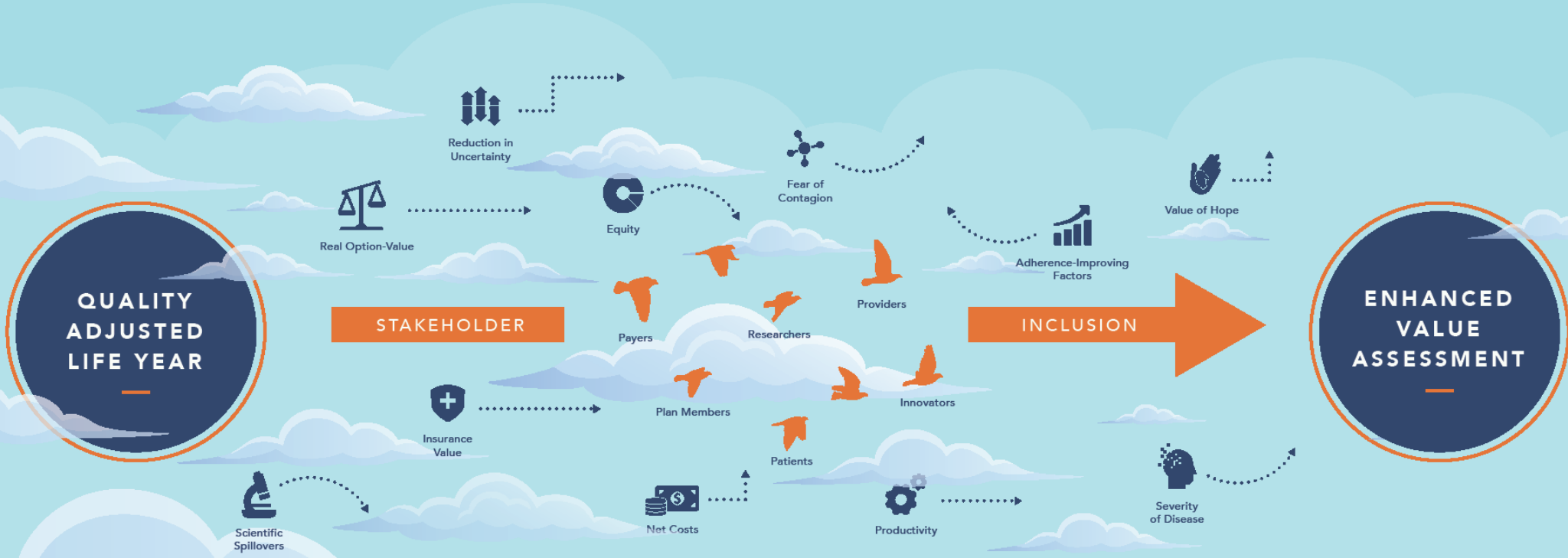
Perspective in published Cost/QALY studies through 2018 (n=6,907)



Change over time in perspective in published CEAs



BROADENING THE COST EFFECTIVENESS RATIO



COST / QUALITY

Thank you!

pneumann@tuftsmedicalcenter.org

Twitter: @PeterNeumann11

The Next Generation of Value Assessment: *Including the Patient Voice*

November 12, 2019

Washington, D.C.- Ronald Reagan Building

Panel #2 Moderated Discussion: Value Assessor Reaction on Why New Methods Are Important and Needed

Moderator:

Sachin Kamal-Bahl, PhD (COVIA Health Solutions)

Panelist:

Steve Pearson, MD, MSc (ICER)

Jennifer Bright, MPA (IVI)

Nicole Mittmann, MSc, PhD (CADTH)

Keynote Speaker

Josephine P. Briggs, MD
Interim Executive Director
PCORI





David Sackett

BMJ

Volume 312:71, January 1996

Evidence based medicine: what it is and what it isn't

It's about integrating individual clinical expertise and the best external evidence

David L Sackett, William M C Rosenberg, J A Muir Gray, R Brian Haynes, W Scott Richardson

“The practice of evidence based medicine means integrating *individual clinical expertise* with the best available external clinical evidence from systematic research. ...”

“By individual clinical expertise we mean... the more thoughtful identification and compassionate use of *individual patients’ predicaments, rights and preferences.*”

patients' predicaments

patients' rights

patients' preferences

The Next Generation of Value Assessment: *Including the Patient Voice*

November 12, 2019

Washington, D.C.- Ronald Reagan Building

Panel #3 Moderated Discussion: How Value Assessment Research Translates into Practical Application in the Health Care System

Moderator:

Sachin Kamal-Bahl, PhD (COVIA Health Solutions)

Panelist:

Karl Cooper, Esq. (AAHD)

Leah Howard, JD (NPF)

Tom Parry, PhD (IBI)

Richard Willke, PhD (ISPOR)

Including the Patient Voice: Evolving Methods for Evolving Value Assessments

Eleanor Perfetto, PhD, MS
Senior Vice President
National Health Council



2019 Challenge Award Presentations

Bryan Luce, PhD, MBA
Chairman
Value Assessment Advisory Committee
PhRMA Foundation



2019 Value Assessment Challenge Awards

1st Prize - Optimizing Representativeness and Enhancing Equity through Patient-Engaged Healthcare Valuation

Lori Frank, PhD and Thomas W. Concannon, PhD,
RAND Corporation

2nd Prize - Expanding Use of Multi-Criteria Decision Analysis for Health Technology Assessment

Charles E. Phelps, PhD, University of Rochester

3rd Prize (tied) Using Patient Experience Data and Discrete Choice Experiment to Assess Values of Drugs

Surachat Ngorsuraches, PhD, Auburn University

3rd Prize (tied) A New Method to Incorporate Uncertainty into Healthcare Technology Evaluations

Darius N. Lakdawalla, PhD, USC and
Charles E. Phelps, PhD, University of Rochester

Patient-Engaged Healthcare Valuation

Lori Frank and Thomas W. Concannon
*The Next Generation of Value Assessment:
Including the Patient Voice*

Washington, D.C.
12 November 2019



HEALTH CARE

Patient-Engaged Healthcare Valuation

Goal: Incorporate the full range of relevant perspectives into healthcare valuation.

Methods:

1. Establish infrastructure
2. Capture goals and prioritization
3. Use those goals and criteria in decision analysis

Key features of the strategy

MCDA by way of GAS:

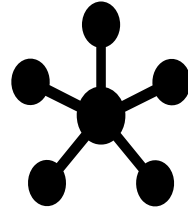
- Captures comprehensive set of criteria for decision analysis
- Decision makers help with weighting criteria

This strategy moves beyond the generic “patient” and connects clinicians and patients via goal attainment scaling.

Goal Attainment Scaling at Scale

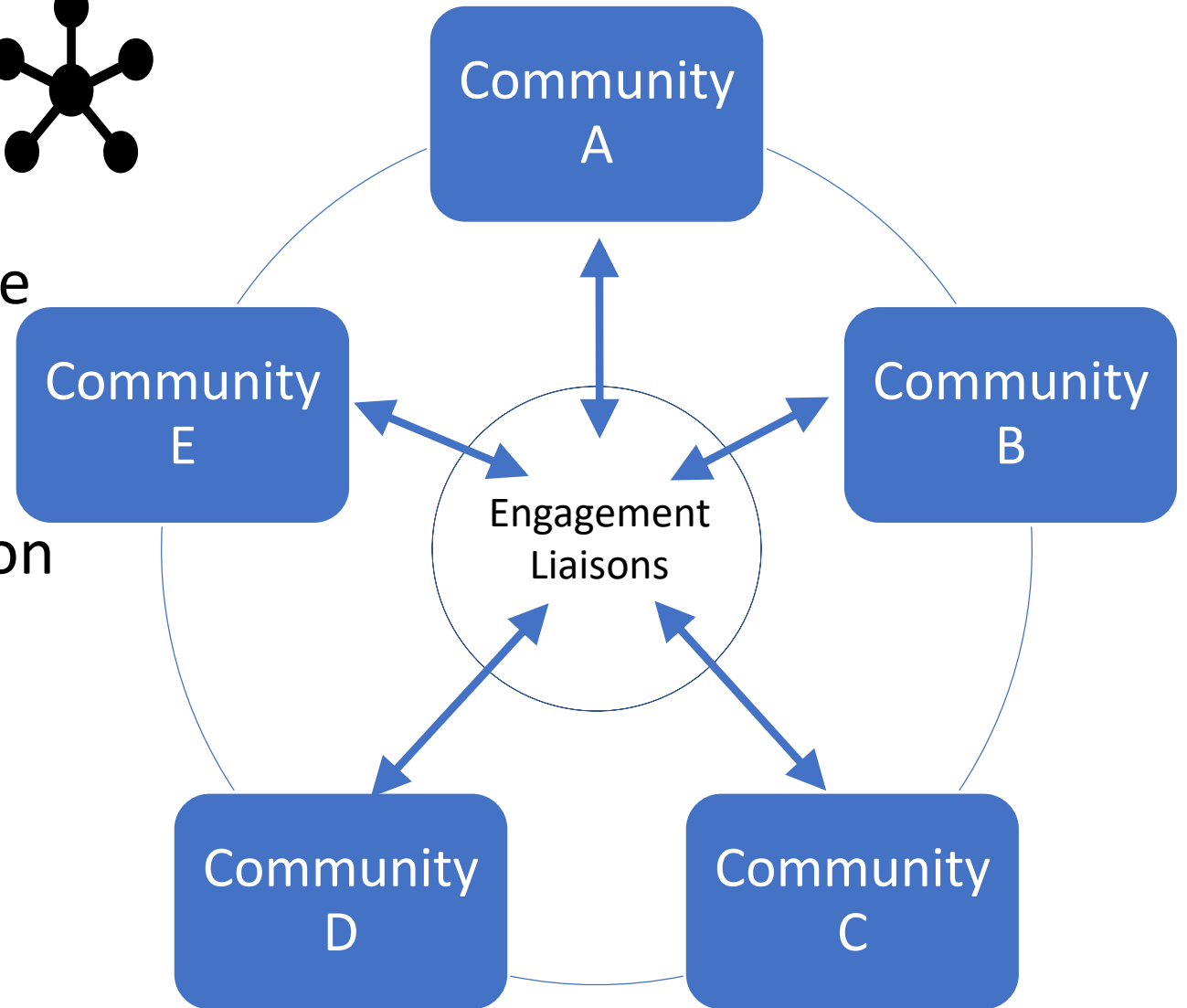
1. **Individual scaling** can be aggregated for goal “saturation.”
2. **Patient panels** create orderly adjudication of goals.
3. **“Multi-channel” goal and scaling** across large samples enables wide reach, including to under-represented communities

“Active Person” Panels



Existing patient communities become **engagement liaisons**

Trained to facilitate goal identification and criteria prioritization



THANK YOU!

Lori Frank, PhD and Thomas W. Concannon, PhD

Contact: LFrank@RAND.org



@LoriBethFrank



HEALTH CARE

Expanding Use of Multi-Criteria Decision Analysis for Health Technology Assessment

(MCDA for HTA)

Charles E Phelps, PhD
University Professor and Provost Emeritus
University of Rochester

Cost Effectiveness – Correct But Incomplete

- Grounded in economic logic
- Measures“ efficiency” using \$/QALY
- The *de facto* standard for comparing medical interventions
- But it’s incomplete
 - Equity/fairness
 - Rare diseases
 - Special populations
 - Scientific spillovers
 - Dread diseases (ebola, zika, AIDS, leprosy, ...)
 - Other

Multi-Criteria Decision Analysis To The Rescue!!!

- Formally includes these “other issues”
- Value measures are unique to decision-maker
 - Different points of view lead to different valuations
- Decision-maker decides what’s important
 - And by how much – the “weights”
- Each alternative scored: How well do they perform on relevant dimensions of value?
- Final scores are weighted sums of performances on value dimensions



You can use the same idea at multiple levels

- Individual patient choices
 - What cancer therapy to accept?
 - Including palliative care
 - What health plan to join?
- Health care provider organizations
 - New technology choices
- Health insurance plans/national systems
 - Coverage decisions about new technologies
- Pharmaceutical manufacturers
 - R&D choices



Reasons for Excitement

- Transparency
- “Flight simulator” testing
- Guides data improvement
- Can improve decision convergence
- Avoids cognitive biases
 - Estimation and use of probabilities
 - Do I already “own” it? If so, its value goes up a lot



Reasons for Concern

- Requires too much data
- Too easy to manipulate
- Each person's index differs
 - what do they mean?
- Too complicated to use and understand
- Can't use with budget constraints



Leading the Way

- Build the data bases
- Reduce user complexity
- Improve for group use
- Create easy-to-use methods in clinical settings
- Education – train students in MCDA as well as CEA
- Use it, use it, use it



Some Sage Advice

“You never change things by fighting against the existing reality. To change something, build a new model that makes the existing model obsolete.” (Buckminster Fuller)

“On the plains of hesitation
Bleach the bones of countless millions,
Who, at the dawn of victory
Sat down to wait, and waiting.....died!”
(George W. Cecil)



Thank You For Your Attention



Using Patient Experience Data and Discrete Choice Experiment to Assess Values of Drugs

Surachat Ngorsuraches, PhD
Auburn University



Inspiration

VIEWPOINT

Peter J. Neumann, ScD
Center for the
Evaluation of Value and
Risk in Health, Institute
for Clinical Research
and Health Policy
Studies, Tufts Medical
Center, Boston,
Massachusetts.

Joshua T. Cohen, PhD
Center for the
Evaluation of Value and
Risk in Health, Institute
for Clinical Research
and Health Policy
Studies, Tufts Medical
Center, Boston,
Massachusetts.

QALYs in 2018—Advantages and Concerns

The quality-adjusted life-year (QALY) is a health metric some people love to hate. Concerns include that QALYs are not patient focused,¹ may be used as rationing tools by health insurers, and may be perceived as dehumanizing. The Affordable Care Act prohibits the Patient-Centered Outcomes Research Institute from using cost-per-QALY benchmarks. The use of QALYs by policy makers to inform coverage and reimbursement decisions is controversial.

However, QALYs are simply a metric to quantify health. Despite concerns, QALYs endure because they help address a difficult and unavoidable question: how to estimate and compare the benefits of what are often heterogeneous health interventions. Recently, QALYs have received increased interest in the United States from the work of the Institute for Clinical and Economic Review (ICER), a private, nonprofit organization that evaluates pharmaceuticals and

Calculation of cost-effectiveness ratios using QALYs also facilitates characterization of intervention value by making it possible to compare those ratios with common benchmarks. Typical value benchmarks in the United States have historically ranged from approximately \$50 000 to, more recently, as high as approximately \$150 000 per QALY.² Those benchmarks purport to represent the “value” of a QALY; ie, the “willingness to pay” to gain 1 QALY of health. The benchmark could also be conceived as a measure of opportunity cost in terms of the health outcomes of the marginal intervention that must be relinquished to provide resources for a new intervention.³ Interventions with lower cost-effectiveness ratios below the benchmark are said to have favorable value because they “buy” QALYs relatively inexpensively; ie, at a cost below the value

Inspiration

Pharmacology Focus

Value-Based Pricing of Disease-Modifying Therapies
for Multiple Sclerosis: Is This Mission Possible?

By Surachat Ngorsuraches, PhD

Inspiration



ELSEVIER

Contents lists available at [ScienceDirect](#)

Multiple Sclerosis and Related Disorders

journal homepage: www.elsevier.com/locate/msard



Clinical trial

Patients' preferences and willingness-to-pay for disease-modifying therapies

Natasha Frost^a, Jerome Freeman^b, Diana Brixner^c, Jane Mort^d, James Clem^d,
Surachat Ngorsuraches^{e,*}



Inspiration

FDA Patient-Focused Drug Development Guidance Series for Enhancing the Incorporation of the Patient's Voice in Medical Product Development and Regulatory Decision Making

Subscribe to Email Updates

Share

Tweet

LinkedIn

Email

Print

Development & Approval Process | Drugs

[Clinical Data Summary Pilot
Program](#)

[Drug Development Tools |
DDTs](#)

[Guidance Documents for Drug
Applications](#)

[Laws, Regulations, Policies
and Procedures for Drug
Applications](#)

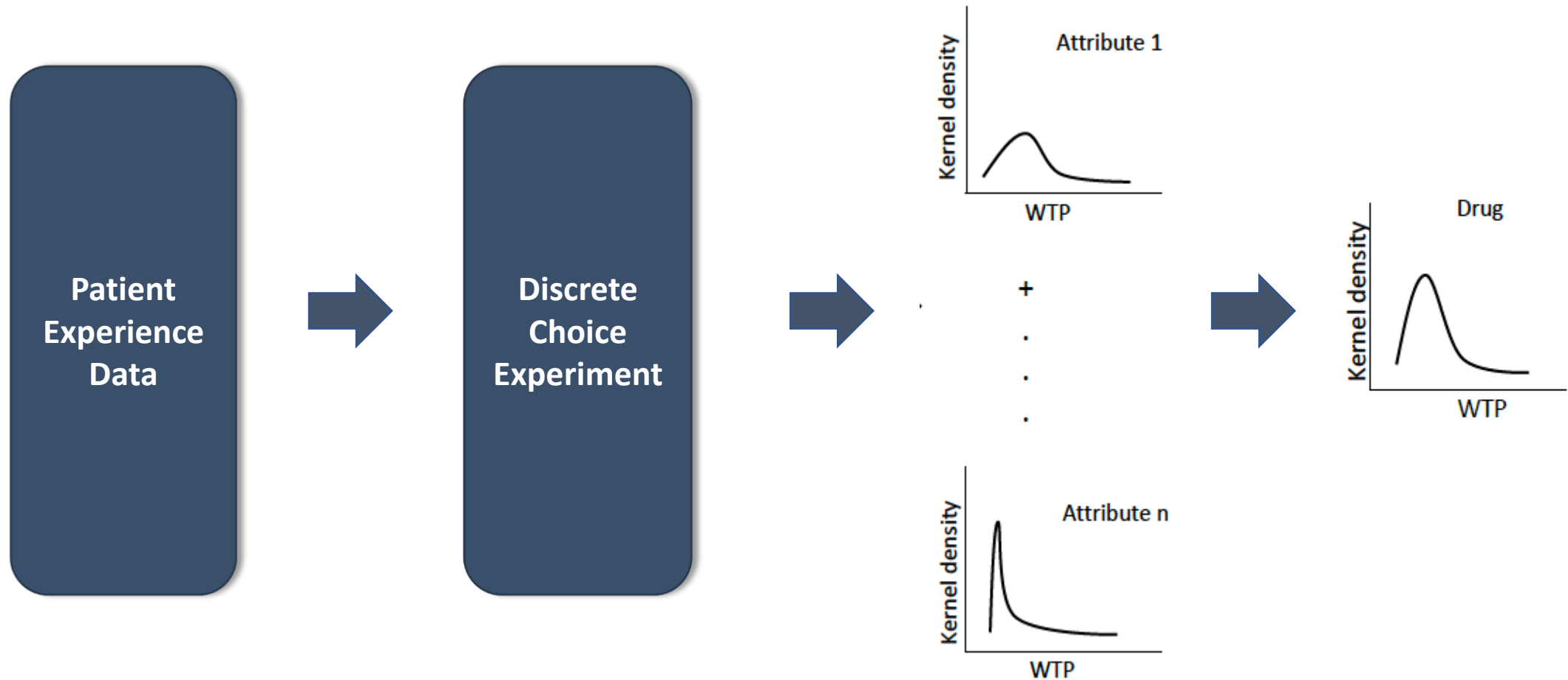


FDA is developing a series of four methodological patient-focused drug development (PFDD) guidance documents to address, in a stepwise manner, how stakeholders can collect and submit patient experience data and other relevant information from patients and caregivers for medical product development and regulatory decision making. This series of guidance documents is intended to facilitate the advancement and use of systematic approaches to collect and use robust and meaningful patient and caregiver input that can better inform medical product development and regulatory decision

Content current as of:
08/29/2019

Regulated Product(s)
Drugs

Patient Experience Framework for Value Assessment



Patient Experience Framework for Value Assessment

Patient-Focused Drug Development: Methods to Identify What Is Important to Patients

Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders

DRAFT GUIDANCE

This guidance document is being distributed for comment purposes only.




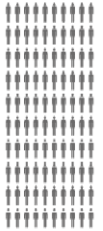




Comments and suggestions regarding this draft document should be submitted within 90 days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit electronic comments to <https://www.regulations.gov>. Submit written comments to the Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number listed in the notice of availability that publishes in the *Federal Register*.

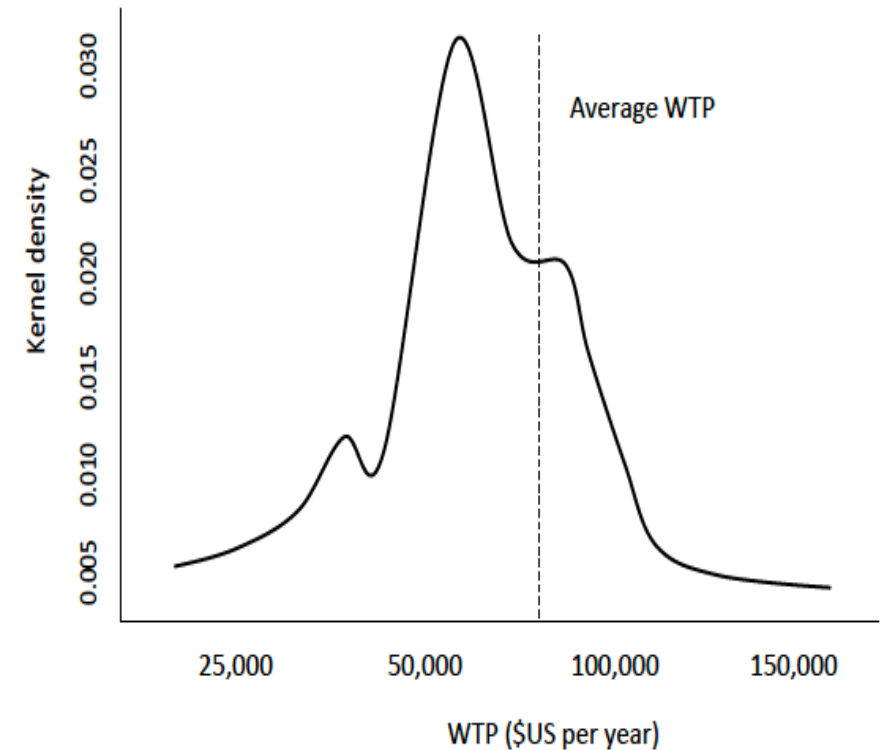
For questions regarding this draft document, contact (CDER) Office of Communications, Division of Drug Information at druginfo@fda.hhs.gov, (855) 543-3784, or (301) 796-3400 or (CDER) Office of Communication, Outreach and Development at ocod@fda.hhs.gov, 800-835-4709 or 240-402-8010.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)

October 2019
Procedural



Treatment Feature	Treatment A	Treatment B	Neither treatment A nor treatment B
Number of relapse in 2 years	2 relapses	No relapse	Neither treatment A nor treatment B
% patients with disability progression in 2 years	0%	15%	
 Patient with disability  Patient with NO disability			
% patients who have severe adverse events	20%	5%	
 Patient with severe adverse events  Patient with NO severe adverse events			
Route of administration	Intramuscular injection	Oral	
Frequency of administration	60 times a month (twice daily)	30 times a month (once daily)	
Out-of-pocket cost per month	\$6,000	\$0	



Images created by iconarray.com. Risk Science Center and Center for Bioethics and Social Sciences in Medicine, University of Michigan. Accessed 2017-01-06

surachat@auburn.edu



A New Method to Incorporate Uncertainty in Health Technology Evaluation

(Adding Uncertainty into HTA)

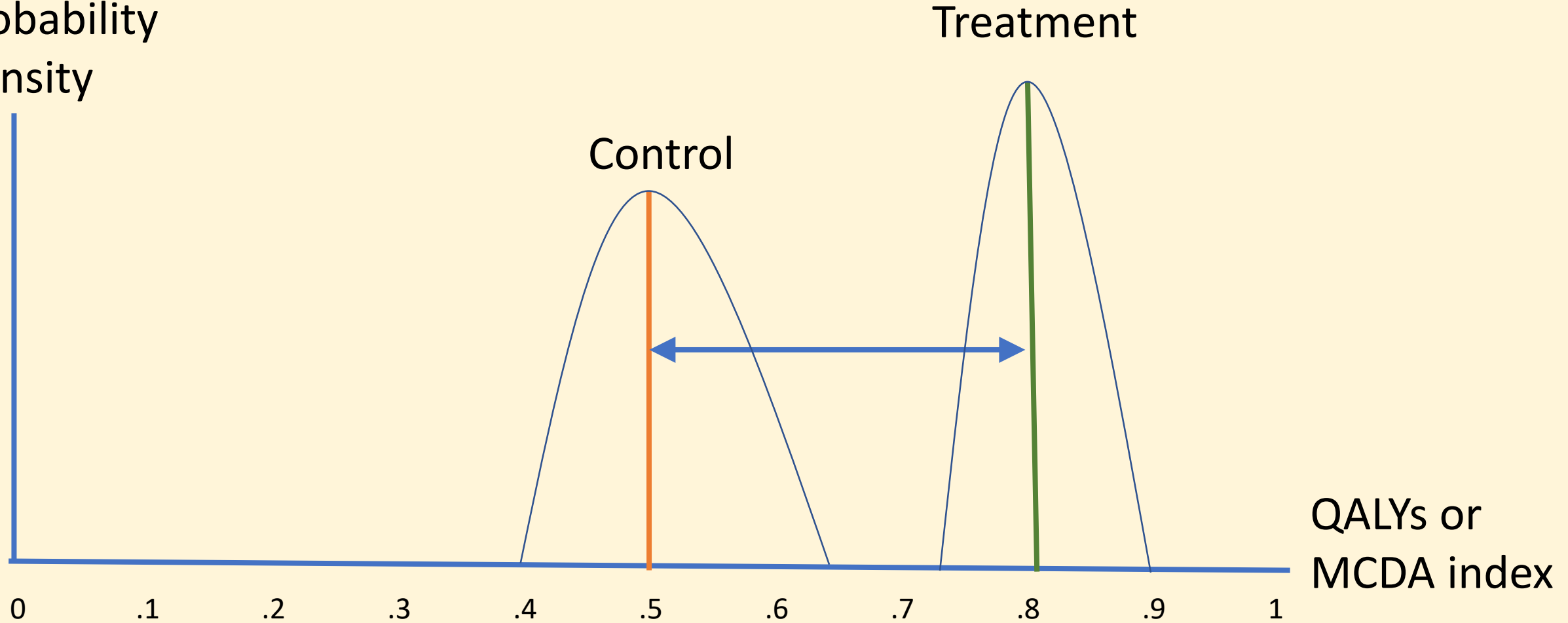
Darius Lakdawalla, PhD
University of Southern California
and

Charles E. Phelps, PhD
University of Rochester



Standard HTA compares mean outcomes

Probability
Density



We add outcomes' uncertainty to the value story

- Just like financial markets measure risk in investment portfolios
- People dislike uncertainty and will pay to reduce it
- That's why people buy insurance
- Less variance is “good”
- More positive skewness is “good”

The math is no fun!

$$\epsilon \approx \left\{ 1 - \frac{1}{2} r^* \left[\frac{\mu_T}{\mu_S} \right] \Delta \Sigma^2 + \frac{1}{6} \pi^* r^* \left[\frac{\mu_T}{\mu_S} \right]^2 \Delta \Gamma_1 \dots \right\}$$



Let's call it the Risk Adjustment Factor (RAF)

$$\epsilon = RAF$$

No, not the Royal Air Force



What do we know about the RAF?

- It measures the relative error from omitting uncertainty
 - $RAF = 1$ mean “no error” in measuring health benefit
 - $RAF = 2$ means true value is 2X what differences of means shows
 - $RAF = 0.5$ means true value is $\frac{1}{2}$ of what differences of means shows
- It matters more when:
 - Average treatment effects are similar
 - Health loss is large
 - Differences in variance are great
 - Differences in skewness are great

You can actually measure this stuff!

- Need to measure variances of outcomes in addition to means
- Also desirable to measure skewness
- If you have big enough samples, add kurtosis (fat tails)

- Combine these with estimates of people's risk attitudes
 - Risk aversion (declining marginal utility)
 - Prudence (declining risk aversion)
 - Temperance (declining prudence)

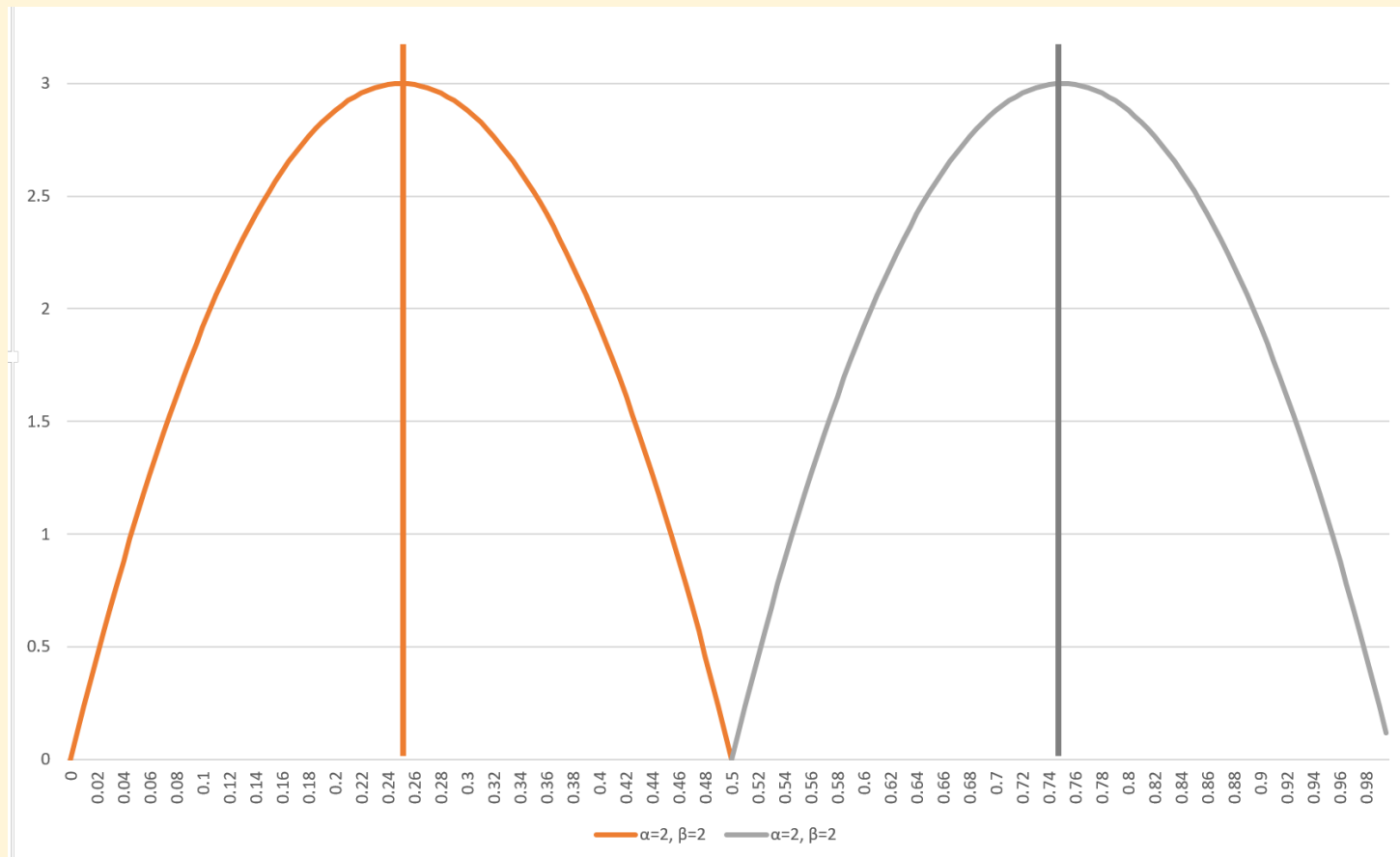
Why RAF Matters

- $ICER_{CORRECT} = ICER_{MEANS} / RAF$
- Example 1:
 - $RAF = 1.3333$
 - ICER using means = \$200,000 per QALY
 - Correct ICER = \$150,000 per QALY
- Example 2:
 - $RAF = .66$
 - ICER using means is \$150,000 per QALY
 - Correct ICER = \$225,000 per QALY



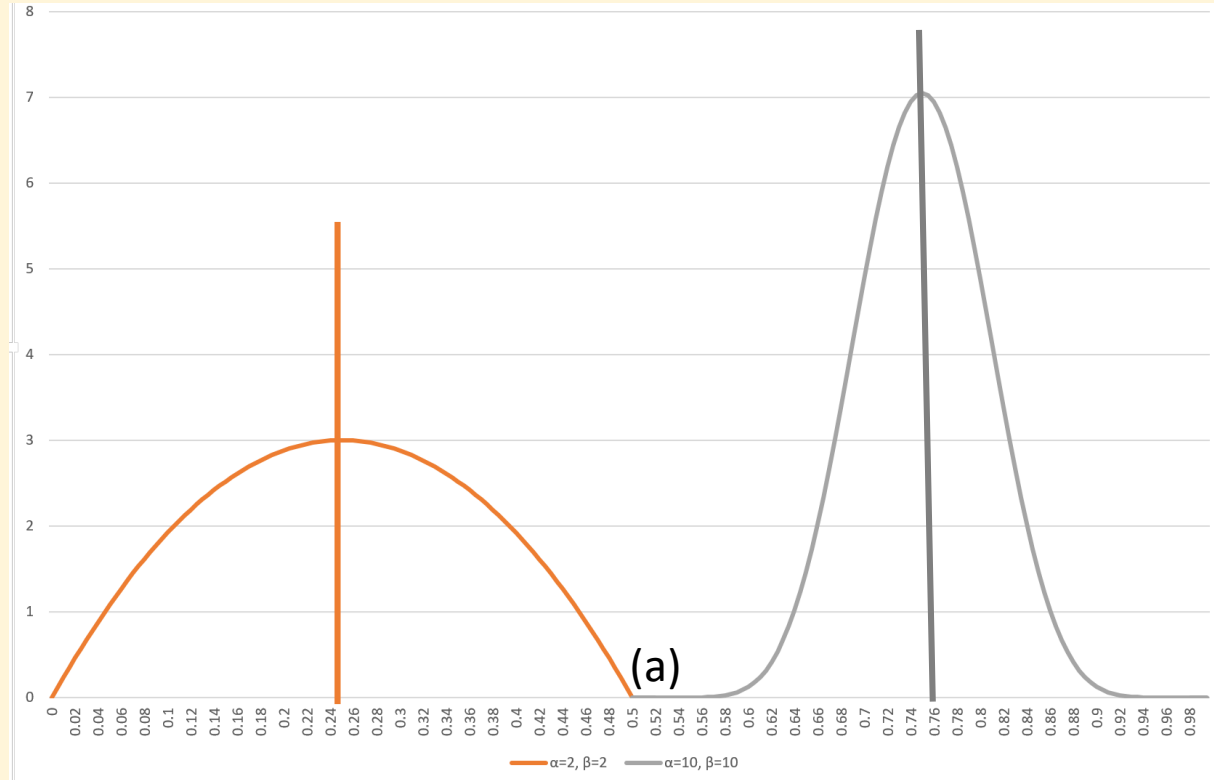
Identical Variances

$$\text{RAF} = 1$$

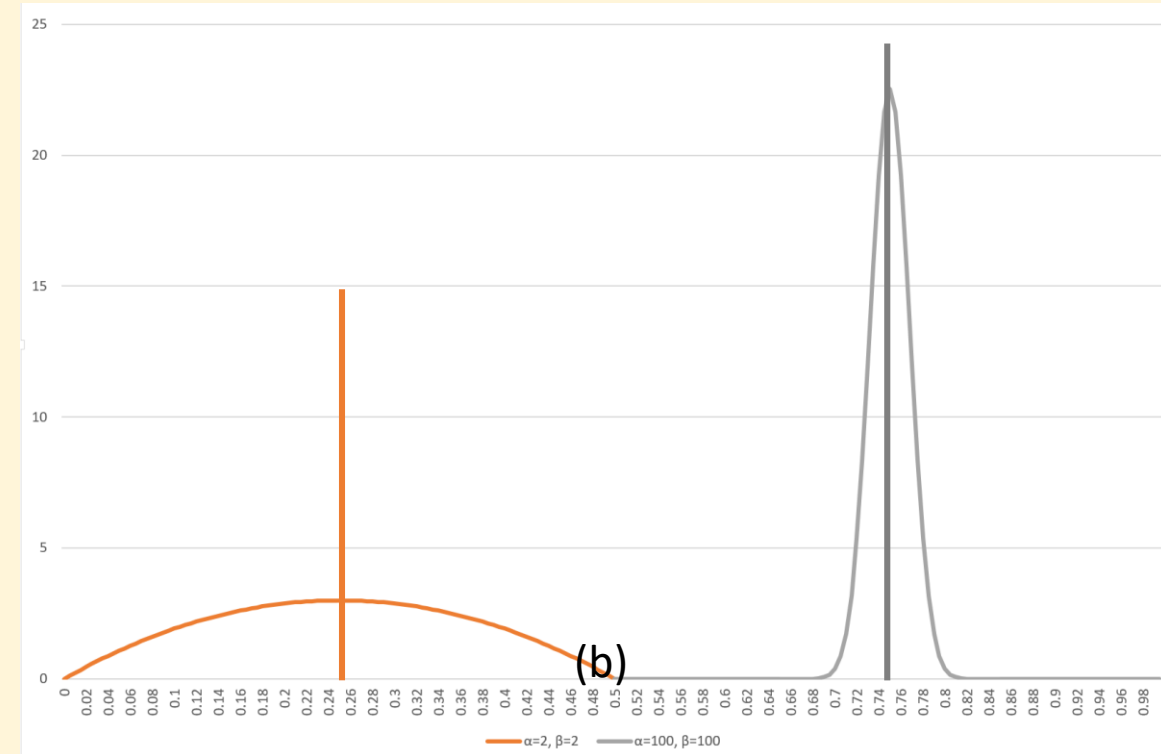


As variance shrinks, value grows

RAF = 1.038



RAF = 1.049

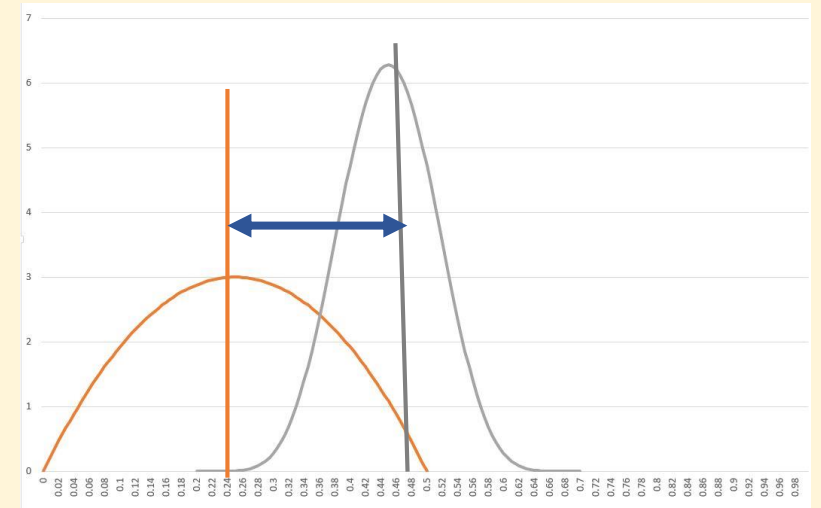
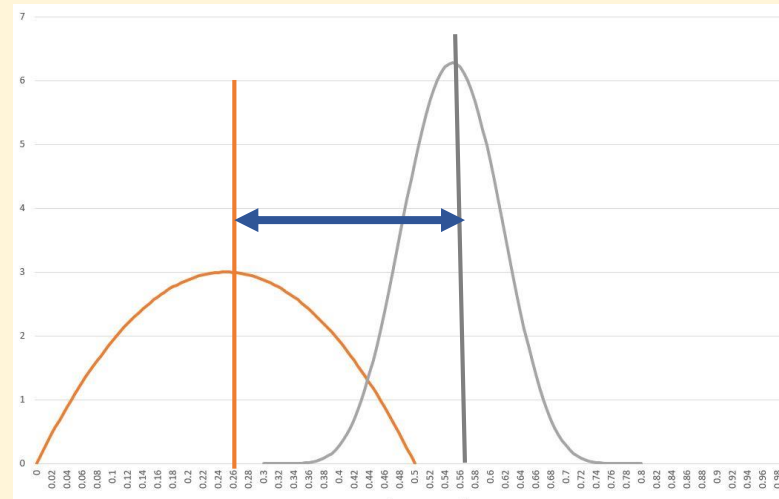
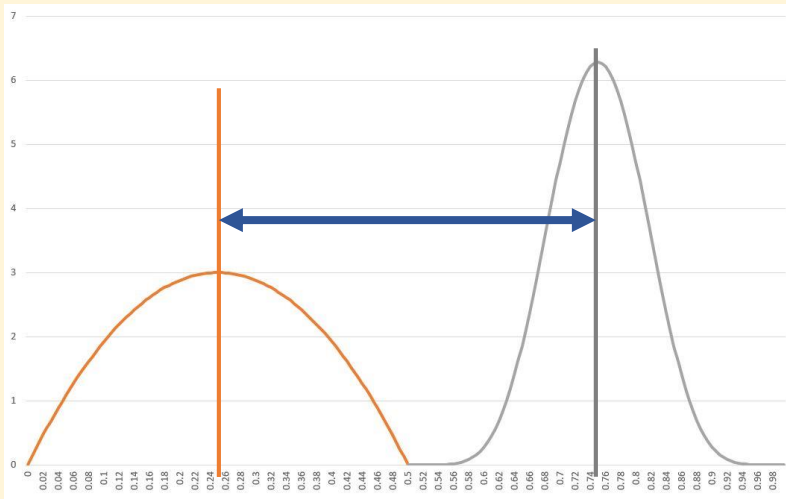


Variance matters more when differences of means are smaller

$RAF = 1.038$

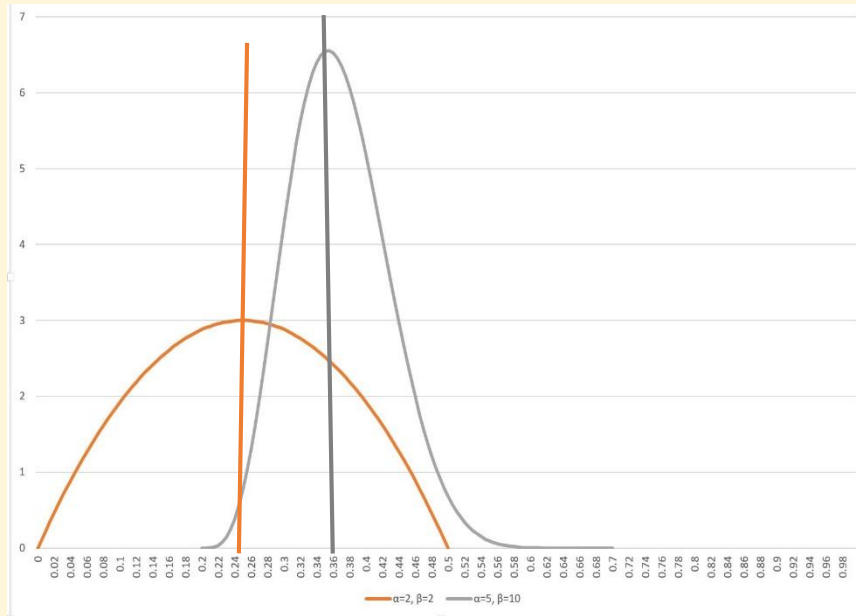
$RAF = 1.10$

$RAF = 1.24$



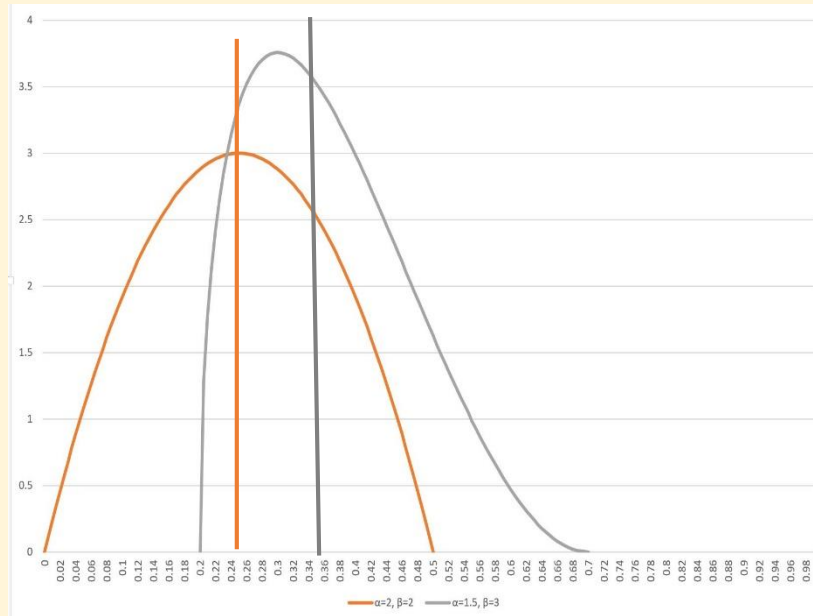
Sometimes even skewness matters a lot

RAF= 1.67



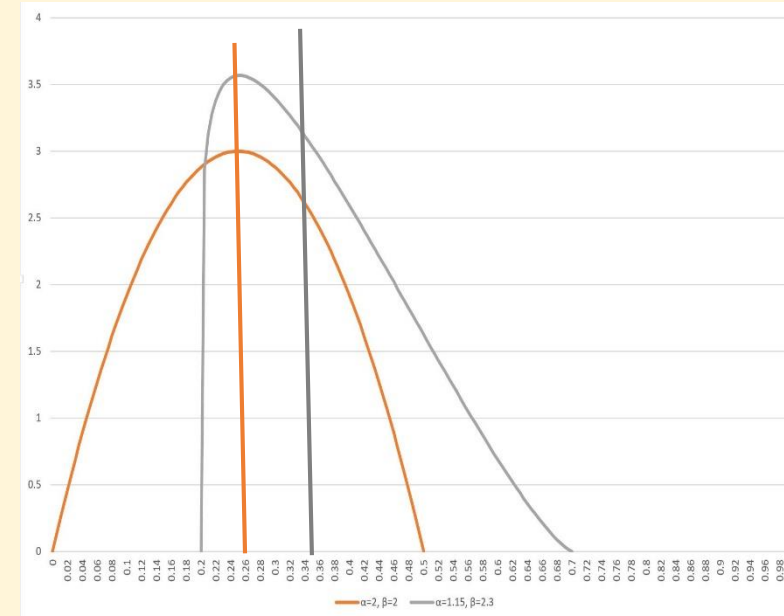
(a)

RAF= 1.22



(b)

RAF = 1.07



(c)

New Data Needed from RCTs (etc.)

- Variances already estimated
 - to measure precision of differences in means
- Skewness never reported, but easy to estimate.
 - Requires bigger “N”
- Kurtosis may be generally irrelevant.
 - Can't know until we look.
 - Requires even bigger “N”

Thank you for your attention!



2019 Value Assessment Challenge Awards

1st Prize - Optimizing Representativeness and Enhancing Equity through Patient-Engaged Healthcare Valuation

Lori Frank, PhD and Thomas W. Concannon, PhD,
RAND Corporation

2nd Prize - Expanding Use of Multi-Criteria Decision Analysis for Health Technology Assessment

Charles E. Phelps, PhD, University of Rochester

3rd Prize (tied) Using Patient Experience Data and Discrete Choice Experiment to Assess Values of Drugs

Surachat Ngorsuraches, PhD, Auburn University

3rd Prize (tied) A New Method to Incorporate Uncertainty into Healthcare Technology Evaluations

Darius N. Lakdawalla, PhD, USC and
Charles E. Phelps, PhD, University of Rochester

Thank you!

Please stay and join us for our reception

