Welcome

Initiative on US Value Assessment Frameworks
Stakeholder Conference
September 23, 2016

Welcome

Initiative on Value Frameworks Stakeholder Conference

Nancy Berg
CEO & Executive Director
ISPOR
Health care today …

Unprecedented Innovation and Change

ISPOR’s Mission

To promote health economics and outcomes research excellence to improve decision making for health globally.
Initiative on US Value Assessment Frameworks

Stakeholder Conference

ISPOR’s Global Constituencies

- Institutional Council
  - Manufacturers
  - CRO/Life Science

- Task Forces
  - Special Interest Groups

- HTA Council
  - HTA Roundtables
  - P&T Roundtables

- Patient Roundtables

- Health Science Policy Council
  - Good Practices for Outcomes Research Reports

- Industry

- Regulators and Assessors

- Decision Makers

Researchers and Academicians

Patients

As Interest in HEOR Has Increased…
ISPOR Membership Has Grown

20,000+ ISPOR Global Members
ISPOR’s Role

Guiding Innovation in Healthcare Decisions

Initiative on US Value Assessment Frameworks

Key methodological issues in defining and applying value frameworks to health care resource allocation

Stakeholder Advisory Panel

Expert Advisory Board

Special Task Force

Stakeholder Conference (Fall 2016)

Themed Issue of Value in Health (early 2017)

Public Call for Papers

White paper and commentaries for publication (2017)

ISPOR’s Initiative on Value Assessment Frameworks was created to inform the shift towards a value-driven health care system in the U.S.
Speakers

Mark McClellan, MD, PhD
Director, Duke Robert J. Margolis, Center for Health Policy

Richard Willke, PhD
Chief Science Officer, ISPOR

Peter Neumann, DSc
Director, Center for the Evaluation of Value and Risk in Health at the Institute for Clinical Research and Health Policy Studies at Tufts Medical Center, and Professor of Medicine, Tufts University School of Medicine

Lou Garrison, PhD
2016-2017 ISPOR President, and Professor, Pharmaceutical Outcomes Research & Policy Program School of Pharmacy University of Washington

Initiative on US Value Assessment Frameworks Stakeholder Conference

Stakeholder Panel

Randy Burkholder
Vice President of Policy and Research Pharmaceutical Research and Manufacturers of America

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

Edmund Pezalla, MD, MPH
Vice President National Medical Director Aetna

Susan Cantrell, RPh, CAE
Chief Executive Officer Academy of Managed Care Pharmacy

Initiative on US Value Assessment Frameworks Stakeholder Conference
Framework Developer Panel

Daniel Ollendorf, PhD
Chief Science Officer
Institute for Clinical and Economic Review

Roger Longman, MA
Chief Executive Officer
Real Endpoints

Lowell Schnipper, MD
Theodore W. and Evelyn G. Berenson Professor of Medicine
Harvard Medical School
American Society of Clinical Oncology

Daniel Mark, MD, MPH
Professor of Medicine
Duke Clinical Research Institute
American College of Cardiology

Mark McClellan, MD, PhD
Director
Duke Robert J. Margolis Center for Health Policy
Washington, DC, USA
Discussion and Q & A

Richard Willke, PhD

Chief Science Officer
ISPOR
Lawrenceville, NJ, USA
Initiative Goals

The Initiative on U.S. Value Assessment Frameworks was created to inform the shift towards a value-driven health care system by promoting the development and dissemination of high-quality, unbiased value assessment frameworks.

Key aspects are to:
• Identify and discuss key methodological and process issues
• Convene a Special Task Force to collaborate on a white paper
• Engage key stakeholders throughout the effort
Key Groups

Steering Committee

- Nancy S. Berg, CEO (ISPOR)
- Jalpa Doshi, PhD (University of Pennsylvania)
- Samantha Dougherty, PhD (PhRMA)
- Lou Garrison, PhD (University of Washington)
- Anthony T. LoSasso, PhD (University of Illinois at Chicago)
- Peter J. Neumann, ScD (Tufts University School of Medicine)
- Joe Vandigo, MS, MBA, PhD Candidate (PhRMA)
- Richard J. Willke, PhD (ISPOR)

- Formed in February 2016
- Responsible for overall coordination of Initiative
- Selected Expert Advisory Board and Stakeholder Advisory Panel

Key Groups

Expert Advisory Board

- Anirban Basu, PhD (University of Washington)
- Josh Carlson, PhD (University of Washington)
- Michael E. Chernew, PhD (Harvard University)
- Patricia M. Danzon, PhD (The Wharton School, University of Pennsylvania)
- Jalpa Doshi, PhD (University of Pennsylvania)
- Michael Drummond, MCom, DPhil (University of York)
- A. Mark Fendrick, MD (University of Michigan)
- Lou Garrison, PhD (University of Washington)
- Henry A. Glick, PhD (University of Pennsylvania)
- Dana Goldman, PhD (University of Southern California)
- Darius Lakdawalla, PhD (University of Southern California)
- Bryan Luce, PhD, MBA (Evidera)
- David Meltzer, MD, PhD (University of Chicago)
- C. Daniel Mullins, PhD (University of Maryland)
- Peter J. Neumann, ScD (Tufts University School of Medicine)
- Mark V. Pauly, PhD (The Wharton School, University of Pennsylvania)
- Charles E. Phelps, PhD (University of Rochester)
- Scott D. Ramsey, MD, PhD (University of Washington)
- Shelby D. Reed, PhD (Duke University)
- Frank A. Sloan, PhD (Duke University)
- Adrian Towse, MA, MPhil (Office of Health Economics)
- Milton C. Weinstein, PhD (Harvard University)
- Richard J. Willke, PhD (ISPOR)

- Formed in April 2016; met in person in May 2016
- Provided key insights into issues regarding current value frameworks
- Helped shape the focal points of the draft Scope of Work
- Will be asked to review draft white paper
Key Groups

**Stakeholder Advisory Panel**

- Formed in May/June 2016; all invited, and many present, here today
- Surveyed for concerns regarding current value frameworks
- Surveyed for comments on draft Scope of Work
- Will be asked to review draft white paper

- Eleanor Perfetto, PhD (National Health Council)
- Sara Van Geertruyden, JD (Partnership to Improve Patient Care)
- Catherine Dave (Familial Hypercholesterolemia Foundation)
- Linda House, RN, BSN, RM (Cancer Support Community)
- Joanna Siegel, SM, ScD (PCORI)
- Amy Miller, PhD (Personalized Medicine Coalition)
- Susan Centrell, MPH (Academy of Managed Care Pharmacy)
- Emily Wilson (American Society for Radiation Oncology)
- Christian Dewire, MHA, JD (Association of Community Cancer Centers)
- Robert Dubois, MD, PhD (National Pharmaceutical Council)
- Newell McElwee, PharmD, MSPH (Merck)
- Sachin Kamal-Bahl, PhD (Pfizer)
- Eleanor Perfetto, PhD (National Health Council)
- Katherine Elkington, PhD (Gilead Sciences)
- Jason Spangler, MD, MPH (Amergin)
- Joseph DiCesare, MPH (Novartis)
- Pratik Shah, MD (Brady-Mays Squibb)
- James Murray, PhD (Eli Lilly and Company)
- Desiree Marks (RTI-HS)
- Nneka Onwudiwe, PharmD, MBA (US FDA)
- Susan Garfield, DrPH, MSc (Ernst & Young)
- Richard Chartier, MSc (Becton Dickinson)
- Andrew Baker, MBA, MPA (Stryker)
- J. Jaime Carr, MCOM (Evidera)
- Mark Sculpher, PhD, MSc (University of York)
- Daniel Malow, PhD, MPH (University of Arizona)
- Andrew Briggs, DPharm, MSc (Memorial Sloan Kettering Cancer Center and University of Glasgow)
- Maarten Ueneman, PhD, MSc (University of Twente)
- Rochelle Henderson, PhD (Express Scripts)
- John Watkins, PharmD, MPH (Premera Blue Cross)
- Andrew Briggs, DPharm, MSc (Memorial Sloan Kettering Cancer Center and University of Glasgow)
- Maarten Ueneman, PhD, MSc (University of Twente)
- Rochelle Henderson, PhD (Express Scripts)
- John Watkins, PharmD, MPH (Premera Blue Cross)

**Special Task Force**

- Formed in July 2016
- Drafted initial Scope of Work
- In-person meetings planned for October & early 2017
- Will write white paper providing guidance on key methodological issues

Lou Garrison, PhD (University of Washington) co-chair
Peter J. Neumann, ScD (Tufts University School of Medicine) co-chair
Ananth Basu, PhD (University of Washington)
Michael E. Chernew, PhD (Harvard University)
Paticia M. Danzon, PhD (The Wharton School, University of Pennsylvania)
Jalpa Doshi, PhD (University of Pennsylvania)
Mark Drummond, MCom, DPharm (University of York)
Darius Lukatavissia, PhD (University of Southern California)
Mark V. Pauly, PhD (The Wharton School, University of Pennsylvania)
Charles E. Phelps, PhD (University of Rochester)
Scott D. Ramsey, MD, PhD (University of Washington)
Adrian Towse, MA, MPhil (Office of Health Economics)
Milton C. Weinstein, PhD (Harvard University)
Richard J. Wilke, PhD (ISPOR), ex-officio
Key Activities

- Call for papers – May 2016
  - 125 abstracts received; 35 invited to submit full papers
    - Included submissions from framework developers & analysts, methodologists, patient groups, and others
  - 32 full papers received in August 2016
  - Manuscript review & revision in progress
    - Many thanks to reviewers from EAB and ViH Board
    - To be published as a themed issue in Jan/Feb 2017
    - Guest co-editors: Jalpa Doshi and Richard Willke

Key Conferences

- ASHEcon, June 2016
  - Introduced this Initiative with both economist and stakeholder perspective sessions

- ISPOR Stakeholder Conference, September 2016
  - Discussion of draft Scope of Work for Special Task Force

- ISPOR Annual International Meeting, May 2017
  - Rollout of final White Paper, following review by our Advisory Boards as well as a primary review group*

* Sign up now by writing to CSO@ISPOR.org
A few borrowed thoughts …

“The curious task of economics is to demonstrate to men how little they really know about what they imagine they can design.”
- Friedrich Hayek

“Every good cause is worth some inefficiency.”
- Paul Samuelson

“Optimistic people play a disproportionate role in shaping our lives. Their decisions make a difference … . They got to where they are by seeking challenges and taking risks.”
- Daniel Kahneman

Peter Neumann, DSc

Director
Center for the Evaluation of Value and Risk in Health at the Institute for Clinical Research and Health Policy Studies at Tufts Medical Center
Professor of Medicine
Tufts University School of Medicine
Medford, MA, USA
Drug Value Frameworks

Presentation at ISPOR
September 23, 2016
Peter J. Neumann, Sc.D.
Tufts Medical Center, Boston
% with “very negative” view

Source: Gallup Poll

Value Frameworks

- AMERICAN COLLEGE OF CARDIOLOGY
- ASCO
- ICER
- National Comprehensive Cancer Network
- Memorial Sloan Kettering Cancer Center
Shifting the Debate on Drug Prices

What does value mean?

R & D Costs

Drug Price

VALUE
Observations

- We should welcome the frameworks
- Private-sector driven
- Scope and audience varies
- This is complicated

Analytic Challenges
What are “attributes” of value?

<table>
<thead>
<tr>
<th></th>
<th>ACA/AHA</th>
<th>ASCO</th>
<th>ICER</th>
<th>Sloan Kettering</th>
<th>NCCN</th>
</tr>
</thead>
<tbody>
<tr>
<td>Clinical benefit</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
</tr>
<tr>
<td>Toxicity/safety</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
<td>X</td>
</tr>
<tr>
<td>Treatment novelty</td>
<td></td>
<td>X</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Condition rarity and condition burden</td>
<td></td>
<td></td>
<td>X</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Budget impact</td>
<td></td>
<td></td>
<td>X</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Cost-effectiveness</td>
<td>X</td>
<td></td>
<td>X</td>
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</tr>
</tbody>
</table>

Other attributes?

Adapted from Garrison, 2016
Whether and how to combine attributes?

Value (common scale?)

American Society of Clinical Oncology (ASCO)
Car Value Framework?

- Safety rating: 5
- Seats: 6
- Miles per gallon: 25

Cost $(thousands):
- $10,000
- $8,000
- $6,000
- $4,000
- $2,000

DrugAbacus

- Actual price
- Drug Abacus price?
- Drug Abacus price?
DrugAbacus

Role of cost-effectiveness analysis?

COSTS

QUALITY-ADJUSTED LIFE YEARS

JAMA | Special Communication
Recommendations for Conduct, Methodological Practices, and Reporting of Cost-effectiveness Analyses
Second Panel on Cost-Effectiveness in Health and Medicine

September 13, 2016
Institute for Clinical and Economic Review (ICER)

Part 1
- Cost-effectiveness analysis
- Care value price

Part 2
- Budget Impact
- Budget threshold
- Health system value price
- Minimum
- Benchmark Value Price

ICER Price Calculations

<table>
<thead>
<tr>
<th>Treatments/Interventions</th>
<th>Price determined by</th>
</tr>
</thead>
<tbody>
<tr>
<td>PCSK9s for high cholesterol</td>
<td>Budget Impact</td>
</tr>
<tr>
<td>Entresto for heart failure</td>
<td>Budget Impact</td>
</tr>
<tr>
<td>CardioMEMS system</td>
<td>Budget Impact</td>
</tr>
<tr>
<td>NASH</td>
<td>Cost-Effectiveness</td>
</tr>
<tr>
<td>Severe eosinophilic asthma</td>
<td>Cost-Effectiveness</td>
</tr>
<tr>
<td>Diabetes</td>
<td>Cost-Effectiveness</td>
</tr>
<tr>
<td>Primary biliary cholangitis</td>
<td>Cost-Effectiveness</td>
</tr>
</tbody>
</table>
Issues with using budget impact

• Penalizes high-value, widely used drugs
• Biases against new technologies
• Short-term horizon

Process challenges

• Transparency
• Stakeholder input
• Updating
What next?

• Value frameworks here to stay?
• What impact?
• What does CMS do?
• ISPOR Task Force

Thanks!

pneumann@tuftsmedicalcenter.org

and www.cearegistry.org
Richard Willke, PhD

Chief Science Officer
ISPOR
Lawrenceville, NJ, USA

Lou Garrison, PhD

2016-2017 ISPOR President
Professor, Pharmaceutical Outcomes Research & Policy Program
School of Pharmacy University of Washington
Seattle, WA, USA
Overview: Special Task Force Statement of Work

Lou Garrison, PhD
Special Task Force Co-Chair
ISPOR President

Initiative on US Value Assessment Frameworks
Stakeholder Conference
September 23, 2016

Background: Motivation

• In the last few years, a number of value assessment frameworks have been developed as the health care system has moved toward a value-driven approach that focuses on evaluating therapeutic options based on health outcomes, value to the patient, and effectiveness compared with other potential treatment options.

• The currently available frameworks, however, are widely diverse in their approaches, and this inconsistency can lead to variable evaluations of treatments.

• A need therefore exists for a robust discussion of relevant perspectives and appropriate approaches that (a) are transparent and methodologically sound and (b) involve the input of key stakeholders to guide the development of value assessment frameworks for health care decision making.
Initiative Aims

1. Identify and discuss key methodological and process issues in defining and applying value frameworks to health care resource allocation issues, and

2. Convene a Special Task Force (STF) to collaborate on a white paper that reviews relevant perspectives and appropriate approaches and methods to support the definition and use of high-quality value frameworks.

3. Engage key stakeholders throughout the development of the white paper to help to frame the scope of work for this methodologically-oriented white paper and to review and comment on the STF’s work progress and products.

Overall Objective of Special Task Force

The Special Task Force (STF) will produce a scientific policy white paper that reviews relevant perspectives and appropriate approaches and methods to support the construction and use of high-quality health care value frameworks that will enable more efficient health sector decision-making in the US.
Incorporating Stakeholder Advisory Panel Feedback

- Formed in May/June 2016; all invited, and many here today
- Surveyed for concerns regarding current value frameworks
- Surveyed for comments on draft Scope of Work
- Will be asked to review draft white paper

Expert Advisory Board (EAB) and Stakeholder Advisory Panel (SAP) Survey Responses

based on 16-18 EAB responses and 25 SAP responses
SAP Question: Do you support the overall aim and methodological focus of the Special Task Force...

“. . . to collaborate on a white paper that reviews relevant perspectives and appropriate approaches and methods to support the definition and use of high-quality value frameworks?”

SAP Question: Version 2.0 of Scope of Work for the Special Task Force...

...is appropriate given the overall aims and timeline of the ISPOR US Value Frameworks Initiative.
Expert Advisory Board survey question:
Do you believe that cost-utility analysis is a valid approach for measuring the value of healthcare interventions?

83% responded “yes” but most noted qualifications:
1. CUA contains limitations or is incomplete (n=5)
2. CUA is one of many possible approaches (n=3)
3. The approach must utilize appropriate measures (n=3)

Key suggested alternatives to CUA included:
- Expanded/extended/enhanced/modified CUA analysis (n=4)
- Value assessment focused on willingness to pay (WTP) (n=2)
- Alternatives to CUA (e.g. multi-criteria decision analysis) (n=2)

EAB and SAP Survey Question:
Which of the following decision-making contexts are the most important for the STF to consider?

Average score (0= least important, 5= most important)

- Payer level (adaptable to the various insurance sectors in the US)
- Societal level (health sector vs. other)
- Patient-physician shared decision making
- Clinical guidelines (physician as agent for broader clinical/societal considerations)
EAB & SAP Survey Question:
Which of the following potential elements of value are the most important for the STF to consider? (part 1)

Average score (0= least important, 5= most important)

- Budget constraints and affordability concerns
- Disinvestment in inefficient technologies
- Excess burden of raising funds via taxation
- Scientific spillovers
- Risk of contagion
- Value of reduction in uncertainty due to dx accuracy
- Value of hope due to the potential for major treatment benefit

SAP responses
EAB responses

EAB & SAP Survey Question:
Which of the following potential elements of value are the most important for the STF to consider? (part 2)

Average score (0= least important, 5= most important)

- Value of peace of mind due to insurance coverage
- Real option value
- Productivity
- Severity of disease
- Near-term mortality probability
- Adherence-improving factors
- Direct input from patients

SAP responses
EAB responses
1. Each of the recent value frameworks has strengths and weaknesses, but those vary by framework; and each has some important limitations.

2. It is important to recognize that the various frameworks have different aims and intended audiences, and they are operating in different health system decision contexts.

3. The value of a medical intervention—from an economic perspective—is reflected in what people are willing to pay or forgo for it.

4. Health gain is generally defined in terms of improvements in either or both mortality or morbidity and is understandably the main driver of value for most healthcare interventions.

5. Cost-utility analysis—based on measuring the quality-adjusted life year (QALY)—has proven to be a very useful tool for comparing health sector interventions; however, it often omits other elements of value that are important in particular circumstances.

6. Expanding the breadth of cost-effectiveness analysis has the potential to capture and reflect some of these other elements of value that can be important for health sector decision-making.
SAP Survey—Feedback on V2.0 SOW

1. Over 80% support the overall aim and methodological focus.
2. Over 80% saw the SOW as appropriate given the overall aims and timeline.
3. All decision contexts were relevant, but payer and shared decision-making are more important to consider.
4. There was broad support for the STF to consider virtually all listed potential elements of value.
5. Highly rated elements to consider included: severity of disease, adherence-improving factors, near term mortality, value of hope, and direct input from patients.

STF—Working Assumptions (1)

1. The effort will focus on reviewing and providing guidance with respect to emerging US value frameworks. Ex-US frameworks and perspectives may be considered, but any science policy recommendations will focus on the US. Of course, the results may be of interest or use to ex-US jurisdictions, but those implications and interactions will be a matter for future discussions.
2. Defining value is important because the US market-oriented healthcare system attempts to signal what is valuable and reward value creation in order to encourage innovation that generates value. Value “signals” can probably be improved in a way that more efficiently encourages the most valued innovations.
3. Most of the emerging value frameworks are being applied to newly launched medicines. In general, value frameworks should be applicable to all types of health technologies to create a level playing field among them. However, new medicines, in particular, but also vaccines and some devices have certain unique public good aspects that may enter into the STF deliberations.

4. Value frameworks have been proposed for different types of decisions or decision contexts: 1) health plan coverage/formulary inclusion (yes/no), 2) payment (i.e., price, tier), 3) clinical guideline development, 4) patient-clinician shared decision-making, 5) regulatory benefit-harm analysis, 6) health sector spending vs. other spending. The STF will need to consider which of these contexts it will address in its deliberations.

Potential White Paper Outline (1)

1. Brief review and comments on existing frameworks, including identification of specific issues that need to be addressed in-depth.

2. Conceptual approach: The need for a conceptual framework for value based on microeconomic principles. In particular, traditional welfare economics, while providing a well-defined framework for decision-making under certain conditions, may not easily accommodate some of the considerations deemed to be relevant in some societies and decision contexts and in some value frameworks. Therefore, the STF may need to address some of the issues surrounding the debate on a “welfarist” vs. “extra-welfarist” approach as it considers expanding the elements of value to be considered.

3. Definition of the scope of this analysis: what contexts and elements of value will be addressed?
4. Is there a common framework? Five aspects—to be repeated and contrasted across the decision contexts—will be addressed:
   a) Defining the decision problem in its specific context, including the decision-makers, their (assumed) incentives, responsibilities, motivation, etc.
   b) Identifying and defining the relevant elements of value
   c) Assessing alternative approaches to measuring the relevant elements of value
   d) Considering how elements of value can be aggregated to estimate full value within the given decision context
   e) Considering how they will be used to reach a decision, e.g., can a threshold be applied, or would a deliberative process be appropriate?

5. Recommended approach: elements, measurement, aggregation, and decision-making

6. Case study examples: applications of recommended approach

7. Recommendations for further research