Qualitative and Quantitative Applications of Incorporating the Patient Voice into Value Assessments

ELISABETH M. OEHRLEIN, PHD, MS

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Solve ME/CFS Initiative
Parent Project Muscular Dystrophy
LEADING THE FIGHT TO END DUCHENNE
NORD
National Organization for Rare Disorders
American Lung Association.
Sjögren's FOUNDATION

NHC Value Classroom:

Support the patient community to meaningfully contribute to value assessment

These resources were partially funded through a Patient-Centered Outcomes Research Institute® (PCORI®) Eugene Washington PCORI Engagement Award (8620-NHC). Development of these resources was also supported by the Patient-Driven Values in Healthcare Evaluation (PAVE) Center, a Center of Excellence supported by a grant from the PhRMA Foundation. Thank you to Allergan, Amgen, Horizon Pharmaceuticals, Novartis, National Pharmaceutical Council, Pfizer, and PhRMA for sponsoring the NHC's work on value.

Health Economics 101

Take our introductory training on health economics and value assessment

Value Assessment Glossary

Plain-language definitions and terms used in value assessment
Click here for an infographic on value terminology

The Patient Voice in Value: The NHC Patient-Centered Value Model Rubric

A tool to help evaluate the patient centeredness of value models and to guide value model developers on the meaningful incorporation of patient engagement throughout their processes

Pearls of Wisdom from Members of the Patient Community

A list of recommendations developed by members of the patient community who have already gone through a value assessment

Value Assessment: Incorporating The Patient Voice

The process of incorporating patient-provided information into a value assessment and how the results might feed into decision-making about care options.

Value Framework Get-Ready Checklist For Patient Organizations

A step-by-step guide for patient advocacy organizations to engage in discussions on and the assessment of value.

Domains of Patient Centeredness in Value Assessment

What are the key characteristics of patient-centered value assessment?

Considerations Guide for Patient Organizations

A tool to assist patient group staff in preparing actionable written comments on a specific value assessment

https://nationalhealthcouncil.org/education/value-classroom/

Patient-Centered Value Assessment

- Patients believe success has been achieved when individual patients sit down with a final VA report and it reflects the multi-dimensionality of their experiences, the burden of disease, and recognizes the different ways a disease manifests
- Desire for better data so patient perspectives can be **quantitatively** incorporated into analyses

*"The **shortage of reliable information on the clinical, humanistic, and economic burden of RDs poses a challenge for accurate assessment of the value and impact of a new RD technology.** The frequently progressive and degenerative nature of RDs, paired with a poor understanding of the disease's natural history, is problematic for HTA modeling and projection of long-term treatment outcomes and associated costs."*

In general, we do not have the data we need to make good decisions

Nestler-Parr S, Korchagina D, Toumi M, et al. Challenges in Research and Health Technology Assessment of Rare Disease Technologies: Report of the ISPOR Rare Disease Special Interest Group. Value Health. 2018;21(5):493–500. doi:10.1016/j.jval.2018.03.004

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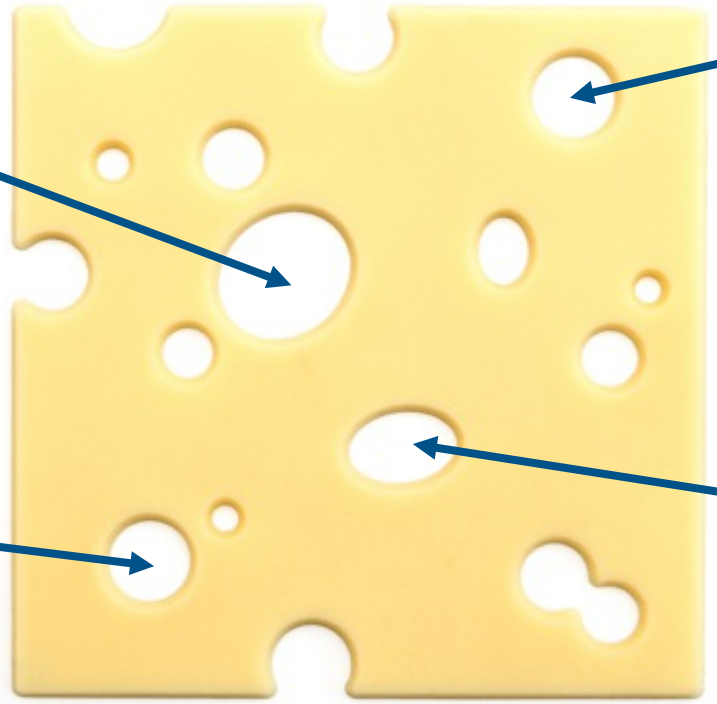
Data on costs that matter to patients are not systematically collected

Out-of-pocket costs to patient and family to access medical services

Costs associated with a family member staying home



Modifications in home as a result of illness

Extra costs for childcare services for caring for un-affected children

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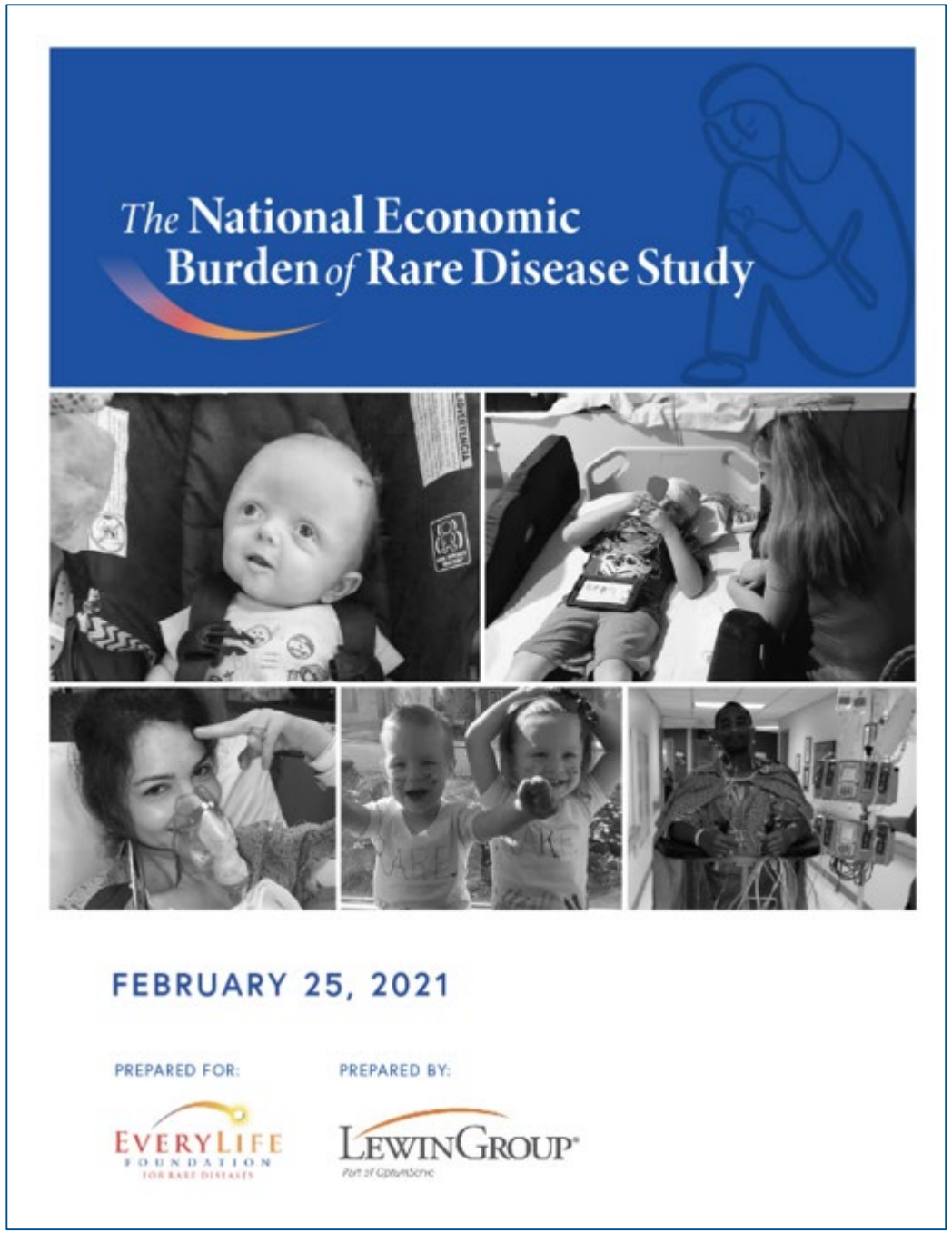
The National Economic Burden of Rare Disease Study



https://everylifefoundation.org/economic-burden-of-rare-diseases-in-the-u-s-approached-1-trillion-in-2019-surpassing-cost-estimates-for-many-chronic-diseases/

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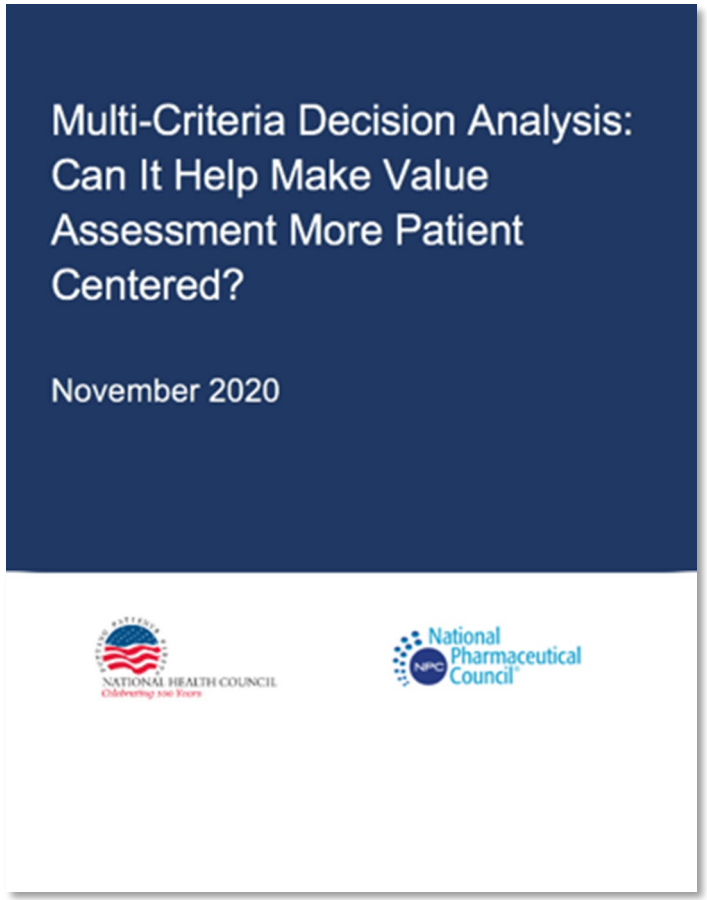
Collaborations to Enhance Patient-Centricity of Value Assessment

Multi-Criteria Decision Analysis: Can It Help Make Value Assessment More Patient Centered?



Key objectives:

- **Socialize what MCDA is**, what it entails, and its potential role in the current landscape of patient-centered V/HTA
- **Demonstrate how to apply MCDA** and how the results can vary based on differing preferences and values
- Identify specific **opportunities to incorporate elements of value** important to patients
- **Review challenges to MCDA uptake** and identify possible solutions to overcome those challenges, **ensuring the patient voice is captured and incorporated** into MCDA-based appraisals.

https://nationalhealthcouncil.org/patient-centered-multi-criteria-decision-analysis/

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What questions patients and employers want researchers to consider as a part of VA for a specific healthcare treatment or service (e.g., drug, device, surgery)?

- What is the intended disease it would be used for? How common is that disease?
- Does it work differently in different people? Does it work particularly well/poorly in certain groups of people? Which subgroups?
- What are the risks or side effects? How common are they?

- How long does it take to work? Are there appreciable differences in short-term vs. longer-term impact on the condition being treated or other related conditions?
- Could an individual's privacy be impacted or infringed?
- What is the cost to patients?

https://nationalhealthcouncil.org/issue/value/

National Alliance of Healthcare Purchaser Coalitions
Driving Health, Equity and Value

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Thank you!

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Patient Engagement in Value Assessment

Melanie D. Whittington, PhD

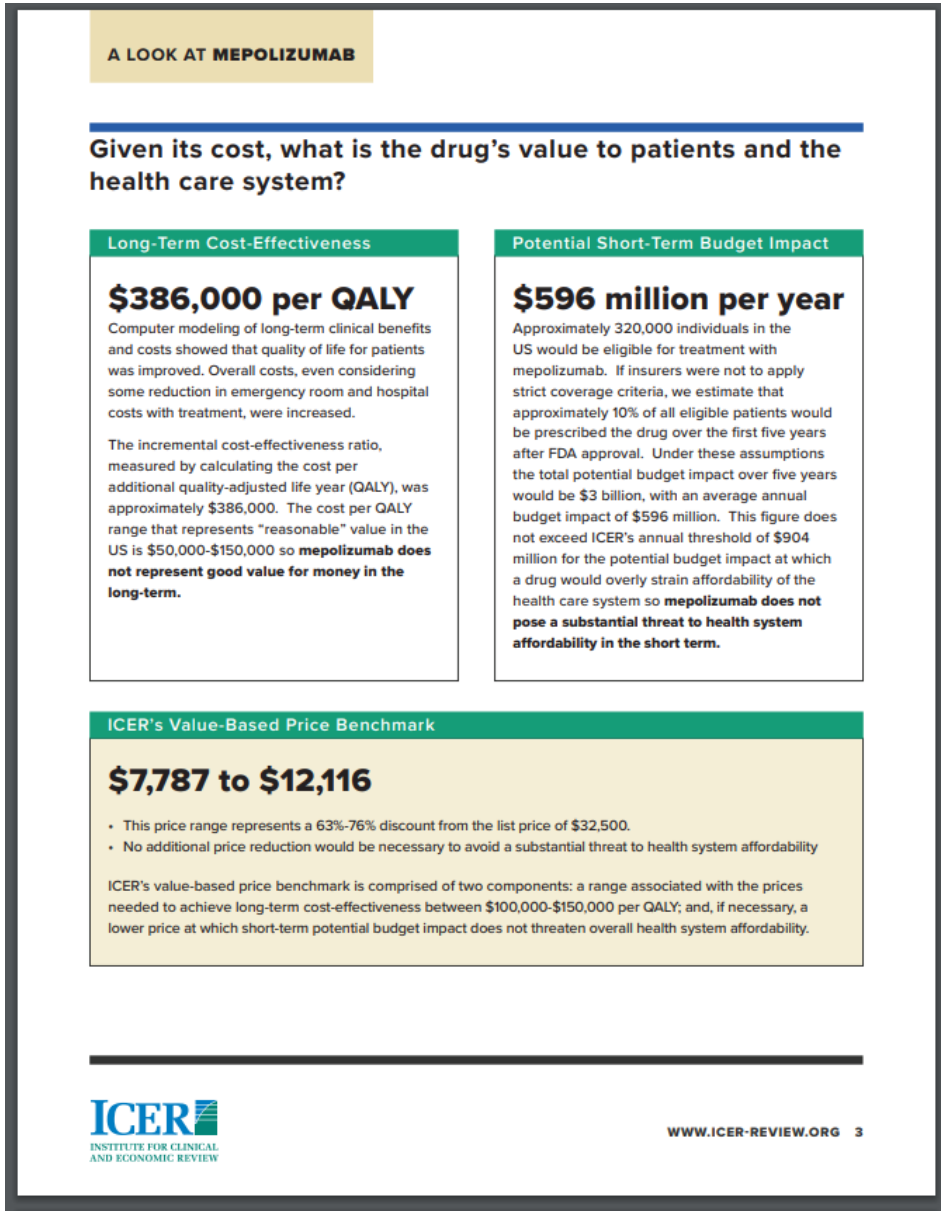
Associate Director of Health Economics

ICER
INSTITUTE FOR CLINICAL AND ECONOMIC REVIEW

Disclosure

ICER's annual policy summit is supported by dues from Aetna, America's Health Insurance Plans, Anthem, Allergan, Alnylam, AstraZeneca, Biogen, Blue Shield of CA, Cambia Health Services, CVS, Editas, Express Scripts, Genentech/Roche, GlaxoSmithKline, Harvard Pilgrim, Health Care Service Corporation, Health Partners, Johnson & Johnson (Janssen), Kaiser Permanente, LEO Pharma, Mallinckrodt, Merck, Novartis, National Pharmaceutical Council, Premera, Prime Therapeutics, Regeneron, Sanofi, Spark Therapeutics, and United Healthcare.

I am not speaking on behalf of ICER. These comments represent my own views.



A LOOK AT **MEPOLIZUMAB**

Given its cost, what is the drug's value to patients and the health care system?

Long-Term Cost-Effectiveness

$386,000 per QALY

Computer modeling of long-term clinical benefits and costs showed that quality of life for patients was improved. Overall costs, even considering some reduction in emergency room and hospital costs with treatment, were increased.

The incremental cost-effectiveness ratio, measured by calculating the cost per additional quality-adjusted life year (QALY), was approximately $386,000. The cost per QALY range that represents "reasonable" value in the US is $50,000-$150,000 so **mepolizumab does not represent good value for money in the long-term.**

Potential Short-Term Budget Impact

$596 million per year

Approximately 320,000 individuals in the US would be eligible for treatment with mepolizumab. If insurers were not to apply strict coverage criteria, we estimate that approximately 10% of all eligible patients would be prescribed the drug over the first five years after FDA approval. Under these assumptions the total potential budget impact over five years would be $3 billion, with an average annual budget impact of $596 million. This figure does not exceed ICER's annual threshold of $904 million for the potential budget impact at which a drug would overly strain affordability of the health care system so **mepolizumab does not pose a substantial threat to health system affordability in the short term.**

ICER's Value-Based Price Benchmark

$7,787 to $12,116

- This price range represents a 63%-76% discount from the list price of $32,500.
- No additional price reduction would be necessary to avoid a substantial threat to health system affordability

ICER's value-based price benchmark is comprised of two components: a range associated with the prices needed to achieve long-term cost-effectiveness between $100,000-$150,000 per QALY; and, if necessary, a lower price at which short-term potential budget impact does not threaten overall health system affordability.

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WWW.ICER-REVIEW.ORG 3

The goal is to have independent information available to catalyze the kinds of discussions that should be happening in broad daylight—because they involve difficult decisions that involve patients—and not behind closed doors.

Steve Pearson, MD
Wall Street Journal
November 4th, 2019

Objectives

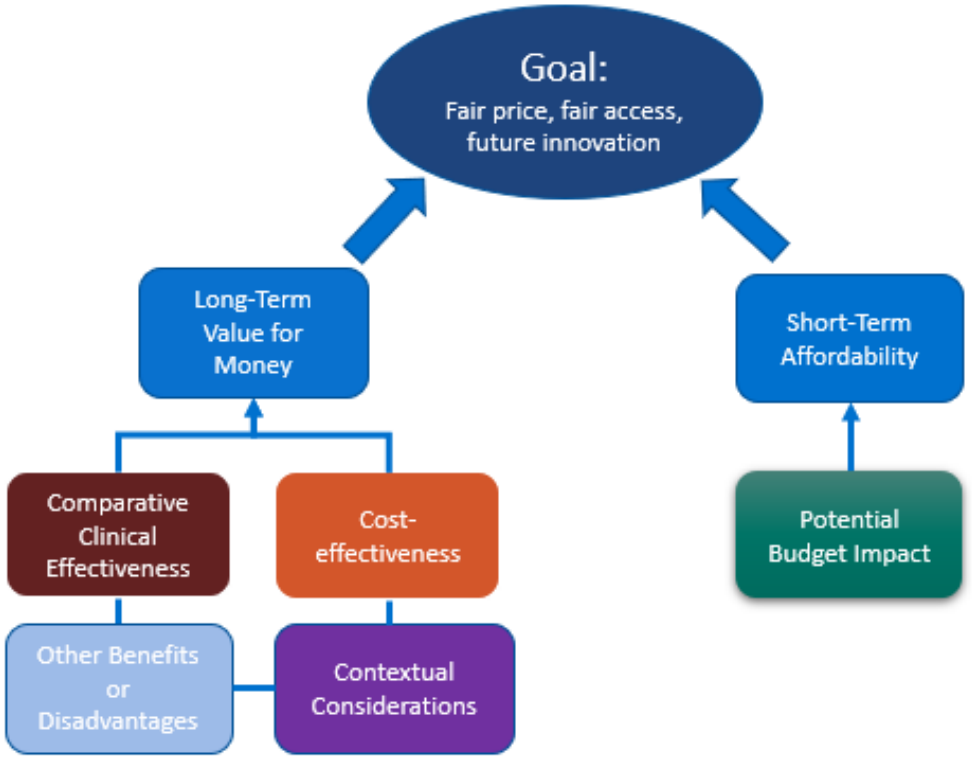
- Detail ICER's Patient Engagement Program
- Identify ways to incorporate the patient perspective in:
  - the cost-effectiveness model
  - the value assessment
  - policies

ICER: Who Are We?

- Independent, non-profit **value assessment group**
- Use **evidence** in a **transparent** way to **align prices with the benefits** for patients and families
- **Improve access and affordability** while retaining the incentives necessary for future innovation

Our Value Assessment Framework (2020-2023)

- Takes a **population-level perspective**
- Within its population-level focus, ICER's value framework seeks to **encompass and reflect experiences and values of patients**



Patient Engagement in a Review

- Engagement initiated before review is announced
- Input into review scope – population, interventions, comparators, outcomes
- Advise on benefits and contextual considerations
- Enhance evidence base - existing or new surveys
- Review preliminary economic model, draft report, draft voting questions
- Patients at entire public meeting, offer public comment, and contribute to the policy roundtable
- Partner on letters to policy makers to address recommendations of the report

Patient Data in the Health Economic Model

- Model structure decisions
- Clinical outcome selection
- Inputs on patient and caregiver preferences
- Inputs on patient and caregiver productivity

Patient Data in the Value Assessment

- Other benefits
  - Complexity
  - Equity
  - Caregiver or family impact
  - Novel mechanism of action
  - Productivity
- Contextual considerations
  - Condition of high severity
  - Lifetime burden of illness
  - First treatment option
  - Uncertainty

Patient Data in Policy Making

- Access to treatments
- Treatments that improve things that are important to patients
- Generation of patient-relevant data

Questions?

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Eliciting, Prioritizing and Quantifying Value Elements from the Patient Perspective

Julia F. Slejko, PhD
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PAVE

PATIENT-DRIVEN
VALUES *in* HEALTHCARE
EVALUATION

Disclosure

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PAVE Team: Laura Bozzi, MS, Yoon Duk Hong, PharmD
Partner: COPD Foundation

Patient-Driven Value Element Elicitation

Our Approach

- A **bottom-up approach** using a systematic process to identify **patient-driven value elements** that are important in healthcare decision-making
  - **Engage** patients directly to elicit elements of value
  - **Prioritize and refine** value element priorities specific to specific clinical areas
  - **Quantify** patient-informed value elements for **translation** to economic evaluation

Patient-Informed Value Elements

The Patient - Patient-Centered Outcomes Research
https://doi.org/10.1007/s40271-020-00433-8

ORIGINAL RESEARCH ARTICLE

Stakeholder-Engaged Derivation of Patient-Informed Value Elements

Susan dosReis[1] · Beverly Butler[2] · Juan Caicedo[3] · Annie Kennedy[4] · Yoon Duk Hong[1] · Chengchen Zhang[1] · Julia F. Slejko[1]

Abstract

Objectives Our objective was to identify patient-informed value elements that can be used to make value assessment more patient centered.

Methods Mixed methods were used iteratively to collect and integrate qualitative and quantitative data in a four-stage process: identification (stage 1), prioritization (stage 2), refinement (stage 3), and synthesis (stage 4). Qualitative methods involved one-on-one discussions with 14 patient stakeholders from diverse medical communities representing mental health, osteoporosis, blindness, lupus, eczema, oncology, chronic obstructive pulmonary disease, and hypercholesterolemia. Stakeholders completed guided activities to prioritize elements important to patient healthcare decision making. Responses were summarized descriptively as frequencies and proportions.

Results Stakeholders identified 94 value elements in stage 1. Of these, 42 elements remained following the stage 2 prioritization and the stage 3 refinement. During the stage 4 synthesis, the 42 patient-informed value elements comprised the principal set of value elements that were organized by 11 categories: tolerability, disease burden, forecasting, accessibility of care/treatment, healthcare service delivery, cost incurred on the patient, cost incurred on the family, personal well-being, stigma, social well-being, and personal values. The categories fell under five domains: short- and long-term effects of treatment, treatment access, cost, life impact, and social impact.

Conclusions In total, 75% of the value elements in the conceptual model were patient derived and distinct from the elements used in existing value frameworks. Recommendations for tailoring, quantifying, and applying the patient-informed value elements in distinct patient communities are provided. This provides a foundation from which future research may test patient-informed value elements in existing value frameworks and economic evaluations.

- The PAVE Center developed with patient input a set of condition-agnostic value elements that are important to patients.

Key Points for Decision Makers

Value assessment framework recommendations call for improving value measures to better align with what is important to patients.

This paper presents patient-informed value elements that were developed with continuous patient engagement throughout the process.

The work will advance the field of value assessment because it provides a set of novel and measurable patient-informed value elements that can be incorporated into existing value frameworks and economic evaluations to improve the health technology assessment, data-generation, and decision-making processes.

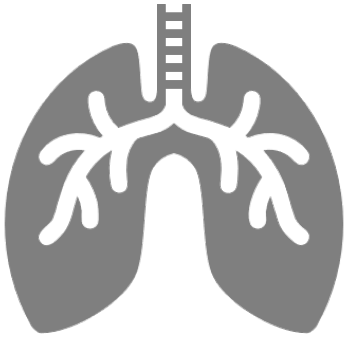
https://rdcu.be/b5Gwg

Value Element Domains

DOMAIN	PATIENT-INFORMED VALUE ELEMENTS		
Short and Long-term Treatment Effects	**Tolerability** Medication Frequency Length of Treatment Side effects	**Disease Burden** Age of Onset Symptoms & Symptom Importance Intermediate/Surrogate Outcomes	**Forecasting** Impact on Education Impact on Career Predictable Healthcare Needs Inability to Plan Life Expectancy
Treatment Access	**Accessibility of Care/Treatment** New Therapeutic Option Available Treatment Provider Willing to Deliver Care Proximity to Care Location Appropriateness of Care System Navigation	**Healthcare Service Delivery** Provider Relationship & Trust Care Transitions Consistency of Care Explanation of Treatment (Risks & Benefits)	
Treatment Costs	**Cost Incurred on the Patient** Affordability Cost of Treatment-Related Side Effects Long-term Costs Reimbursed Care	**Cost Incurred on the Family** Sibling Costs Long-term Effects on the Family Relocation Costs Level of Autonomy/Dependence	
Life Impact	**Personal Well-Being** Fatigue Ability to Work Physical Abilities Emotional Status	**Social Well-Being** Support Network Relationship with Family Relationship with Peers Maintain Social Activities	
Social Impact	**Stigma** Embarrassment/Self-Consciousness Rejection by Family Rejection by Society	**Personal Values** Cultural Barriers Religious Beliefs	

Value Element Prioritization for Chronic Obstructive Pulmonary Disease (COPD)

Goals and Motivation



Motivation

- Economic evaluations of COPD treatments rely on clinical endpoints such as exacerbations and forced expiratory volume (FEV1).
- How do these align with value elements important to patients?

The Patient - Patient-Centered Outcomes Research
https://doi.org/10.1007/s40271-021-00495-2

ORIGINAL RESEARCH ARTICLE

Prioritization and Refinement of Patient-Informed Value Elements as Attributes for Chronic Obstructive Pulmonary Disease Treatment Preferences

Julia F. Slejko[1] · Yoon Duk Hong[1] · Jamie L. Sullivan[2] · Robert M. Reed[3] · Susan dosReis[1]

Accepted: 7 January 2021

Goal:

Identify patient-driven value element priorities among individuals with COPD, to be used in a stated preferences instrument.

Phase I – Value Element Elicitation

- **Four activities + demographic/condition questionnaire**
  - Value element domain importance
  - Treatment-related factors - 15 elements
  - Outcome-related factors - 17 elements
  - Care Process Factors – 10 elements

- **Participants (n=23)**
  - Baltimore-area pulmonary clinics
  - COPD Foundation advocacy events

Slejko JF et al. *Patient - Patient-Centered Outcomes Res.* 2021.
https://rdcu.be/ceVUN

Value Element Prioritization

Value Elements Rated Among the 5 Most Important to Individuals with COPD (n = 23)

Value Elements	Number of Votes
Treatment-related Factors	
Side Effects	15 (65%)
Provider Relationship & Trust	15 (65%)
Medication Frequency	14 (61%)
New Therapeutic Option	10 (44%)
Available Treatment	10 (44%)
Affordability	10 (44%)
Appropriateness of Care	8 (35%)
Long-Term Costs	8 (35%)
Fatigue	8 (35%)
Length of Treatment	8 (35%)
Cost of Treatment-Related Side Effects	5 (22%)
Reimbursed Care	2 (9%)
Embarrassment/Self-Consciousness	1 (4%)
Religious Beliefs	1 (4%)
Cultural Barriers	0 (0.0%)

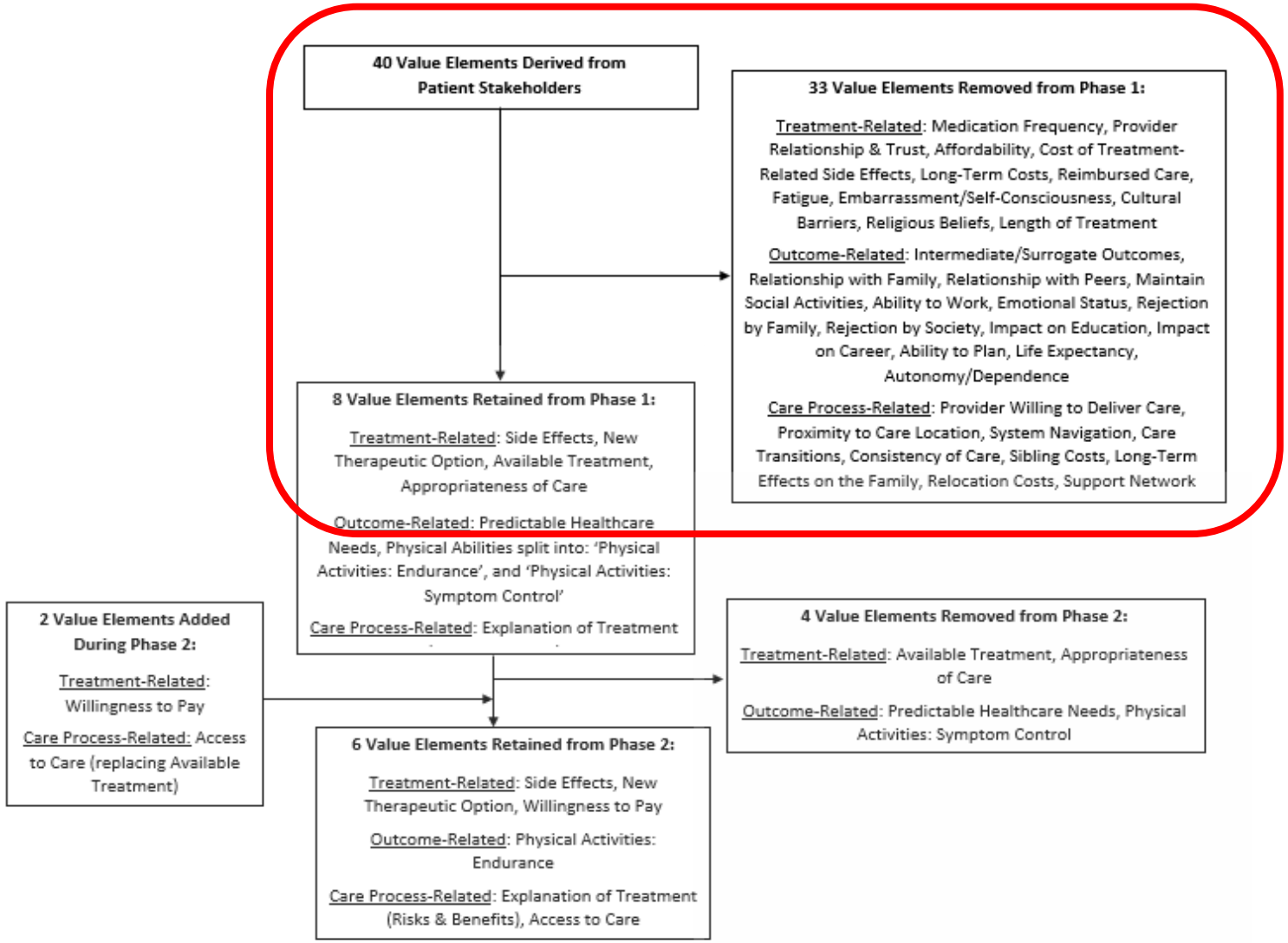
Value Elements	Number of Votes
Outcome-related Factors	
Physical Abilities	22 (96%)
Maintain Social Activities	14 (62%)
Fatigue	12 (52%)
Life Expectancy	9 (39%)
Relationship with Family	8 (35%)
Emotional Status	8 (35%)
Predictable Healthcare Needs	8 (35%)
Ability to Plan	7 (30%)
Intermediate/Surrogate Outcomes	6 (26%)
Autonomy/Dependence	6 (26%)
Rejection by Family	4 (17%)
Ability to Work	3 (13%)
Impact on Education	3 (13%)
Relationship with Peers	2 (9%)
Impact on Career	2 (9%)
Rejection by Society	1 (4%)
Embarrassment/Self-Consciousness	0 (0.0%)

Value Elements	Number of Votes
Care Process Factors	
Explanation of Treatment (Risks & Benefits)	21 (91%)
Provider Willing to Deliver Care	19 (83%)
Proximity to Care Location	14 (61%)
Consistency of Care	14 (61%)
Support Network	14 (61%)
Long-Term Effects on the Family	7 (30%)
System Navigation	5 (22%)
Care Transitions	5 (22%)
Sibling Costs	2 (9%)
Relocation Costs	1 (4%)

-Plus feedback from COPD patient advocate and clinician
-8 Elements remained for Phase II

Slejko JF et al. *Patient - Patient-Centered Outcomes Res.* 2021. https://rdcu.be/ceVUN

Phase I – Element Prioritization

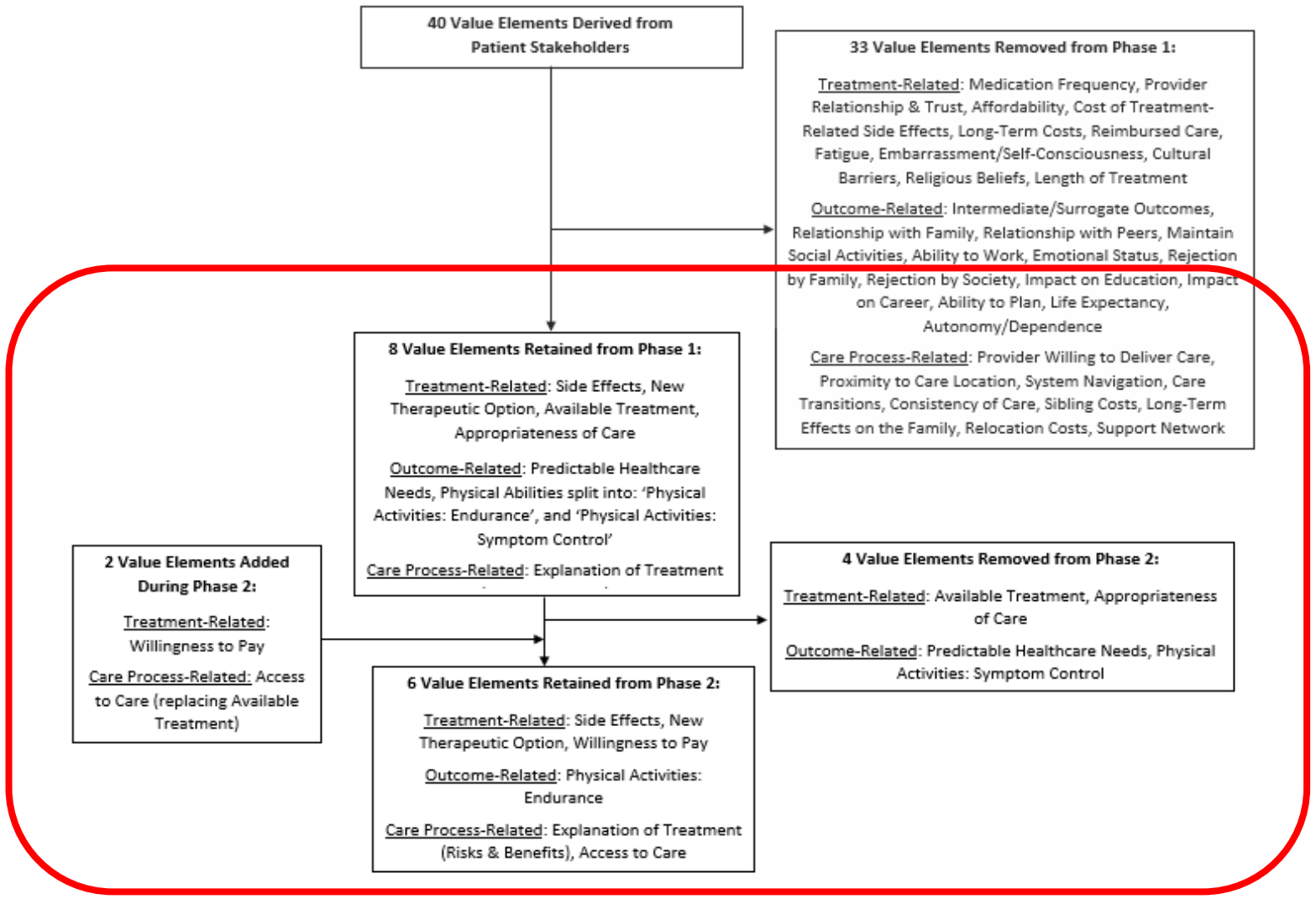


Which elements are most important?

- **8 Elements retained for Phase II**

Slejko JF et al. *Patient - Patient-Centered Outcomes Res.* 2021. https://rdcu.be/ceVUN

Phase II – Element Refinement



Iterative Process:
7 patient experts and advocates

Eliminate redundancy or overlap:
- 4 elements removed, 2 elements added
-6 elements retained for stated preference instrument

Slejko JF et al. *Patient - Patient-Centered Outcomes Res.* 2021. https://rdcu.be/ceVUN

Phase II - Language Refinement

Example of Language Refinement to Identify Statements for the Patient Preference Instrument

Attribute	Explanation of the Attribute to the Patient Experience	Attribute Statement
New Therapeutic Option	Does the treatment replace another one in the regimen?	Reduce/increase the # of meds in regimen
Physical Abilities - Endurance	Does the treatment affect my endurance?	Distance one can walk; walking up stairs;
Physical Abilities - Symptom Control	Does the treatment control symptoms so that I can function in daily activities?	Shortness of breath; staying employed; go to work; fatigue
Side Effects	How will I deal with side effects if they occur?	Change or add medication
Explanation of Treatment Benefits & Risks	Was this the best alternative given an individual's needs?	Alternative options (interventions vs. surgical) & related benefits or risks

How do the important elements relate to the specific condition?

Pilot Stated Preference Instrument

Attribute	Description of Attribute	Level
Side Effects	You manage side effects by	Making no changes to your medicines
		Lowering the medicine dose
		Adding another medicine
Willingness to Pay	You pay out-of-pocket	$30 per month
		$90 per month
		$120 per month
Access to Care	Your doctor replies within	24 hours
		48 hours
		72 hours
New Therapeutic Option	The medicine is taken	1 time per day
		2 times per day
		3 times per day
Explanation of Benefits and Risks	You learn about treatment options	From your provider or pharmacist
		From a patient support group

What description & levels make sense for the specific condition?

Recommendations

- ❑ Partner with a patient organization
- ❑ Recruit diverse patients
- ❑ Leave enough time for an iterative process
- ❑ Let the results guide your next steps

Application of Value Elements

Quantify and Translate COPD findings to economic evaluation

Notions of "Value" in Healthcare

Future of Patients in Healthcare Evaluation: The Patient-Informed Reference Case

Julia F. Slejko, PhD,[1,*] T. Joseph Mattingly II, PharmD, MBA,[2] C. Daniel Mullins, PhD,[1] Eleanor M. Perfetto, PhD, MS,[1,3] Susan dosReis, PhD[1]

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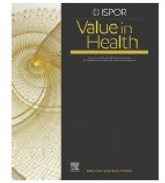

ility among published cost-effectiveness analyses intended to
re resources. Although the societal perspective is recommended
dequately represent the patient perspective, and more often,
nmentary, we discuss the evolution of the Reference Case over
is should begin with a patient-informed societal perspective. A
ent perspectives in CEA creates a conduit for patient inclusion.
ements and prioritize value elements using stated preference
ctive and a patient-informed Reference Case analysis.

ective

Slejko JF et al. *Value in Health*. 2019;22(5):545-548.

Implementing the Patient-Informed Reference Case

Patient-Informed Elements Existing in the Societal Perspective	Novel, Patient-Informed Value Elements
• Patient costs • Costs and impacts of side effects	• Medication frequency • Preferences for provider interaction
Valuation of Health State Utilities	**Patient Engagement in Economic Evaluations**
• Physical abilities adequately captured? • Preference heterogeneity	• Side effects

Questions & Information

Patient Driven Values in Healthcare Evaluation (PAVE) Center:
https://pharmacy.umaryland.edu/PAVE

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Skaggs School of Pharmacy and Pharmaceutical Sciences

UNIVERSITY OF COLORADO
ANSCHUTZ MEDICAL CAMPUS

Patient-Centered Value Criteria for Multi-Criteria Decision Analysis

R. Brett McQueen, PhD, Assistant Professor
Director, Center for Pharmaceutical Value (pValue)

May 17, 2021

pValue
Evidence for Decisions

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- Disclosures:
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pValue
Evidence for Decisions

Conventional Cost-Effectiveness Analysis (CEA)

- Traditional, conventional, or regulatory-anchored CEA evidence standard for generating “value-based” prices
  - Starting point for price negotiations with uptake from some US payer entities[1]
- CEA objective conditioned on assumptions and inputs
  - By definition omits additional criteria potentially influential to value perceptions
- Limitations of CEA and comparative effectiveness has increased focus on other value criteria, i.e., “novel” or “additional” value criteria[2]

1 https://cvshealth.com/sites/default/files/cvs-health-current-and-new-approaches-to-making-drugs-more-affordable.pdf

2 Lakdawalla et al. Defining Elements of Value in Health Care. Value in Health 21 (2018) 131-139

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U.S. Value Interpretations…*It Depends*

- Other criteria influence US value interpretations[3,4,5]
  - Novel mechanism
  - Reduce caregiver burden
  - Lack of evidence
  - Uncertainty in long-term safety
- Despite different perspectives and decision contexts, significant overlap in valuing other novel criteria between payers and patients[6]

3 Neumann PJ et al. Should A Drug's Value Depend On The Disease Or Population It Treats? Insights From ICER's Value Assessments. Health Affairs Blog Nov 6, 2018;
4 Trenaman L, Pearson SD, Hoch JS. How Are Incremental Cost-Effectiveness, Contextual Considerations, and Other Benefits Viewed in Health Technology Assessment Recommendations in the United States? Value Health. 2020 May;23(5):576-584; Lakdawalla et al. Defining Elements of Value in Health Care. Value in Health 21 (2018) 131-139
5 Mendola et al. Stakeholder Perception of Pharmaceutical Value: A Multicriteria Decision-Analysis (MCDA) Educational Case Study for Value Assessment in the United States. *Under Review AND* *https://nationalhealthcouncil.org/wp-content/uploads/2020/11/MCDA-Roundtable_white-paper_final.pdf*
6 Jakab I et al. Criteria for Inclusion in a Multiple Criteria Decision Analysis: Comparing Patient and Payer Preferences for Additional Value Criteria. *Under Review*

Value is Context- and Perspective-Specific

Value Domains	Example Value Criteria	Inclusion in Value Assessment	Perspective
Traditional, conventional, or regulatory-anchored value criteria	• Survival • Quality of life (including safety impact)	• Quantitatively incorporated in CEA/CER	• Payer or societal perspective
Additional or novel societal value criteria	• Insurance value • Rarity • Improved access to vulnerable populations	• Qualitatively incorporated through deliberations on other factors*	• Societal perspective
Additional or novel patient-centric value criteria	• Ability to reach important personal milestones • Patient experience related to disease management • Value of hope	• Qualitatively incorporated through deliberations on other factors*	• Disease-specific patient perspective

*We acknowledge there are ongoing methods improvements in these areas but at this time infrequent inclusion in current quantitative value assessment evaluations

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Inclusion of Additional Value Criteria in Value Assessment

- Improve or replace existing value assessment methods
  - Revise conventional CEA to include other novel value criteria (e.g., augmented CEA)[7,8]
- <u>Supplement existing value assessment methods</u>
  - Quantify and prioritize additional value criteria using MCDA
    - Prioritization or “weight” of value criteria stakeholder-specific

7 Garrison et al. Augmenting Cost-Effectiveness Analysis for Uncertainty: The Implications for Value Assessment – Rationale and Empirical Support. JMCP 2020; vol. 26, no.4
8 Lakdawalla and Phelps. Health Technology Assessment with Diminishing Returns to Health: The Generalized Risk-Adjusted Cost-Effectiveness (GRACE) Approach. ViH 2021; 24(2)

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Pharmaceutical Value (pValue) Goals

- Develop and test qualitative and quantitative multi-criteria decision analysis (MCDA) tools
  - Qualitative disease-specific modified impact inventory tables
  - Quantitative MCDA decision tools
- Improve patient-centricity outside of conventional value
- Value framework perspective

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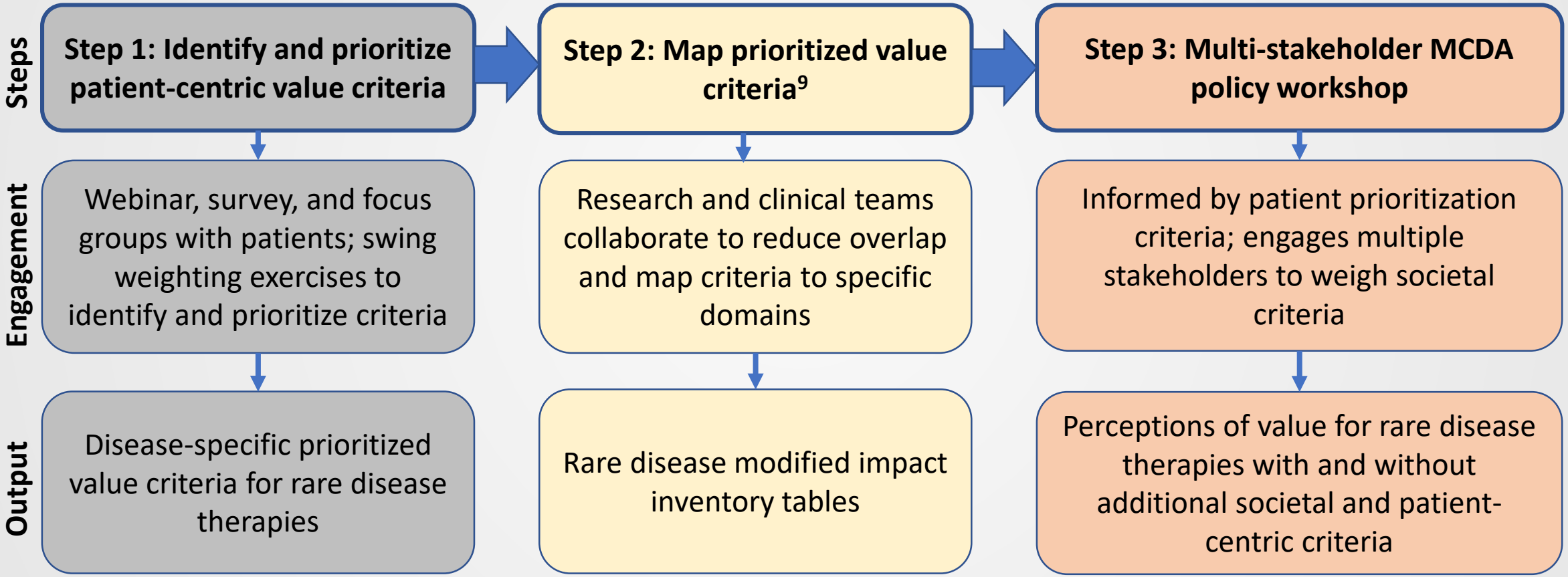
pValue Stepwise Approach to Improving Patient-Centricity in Value Assessment



9 McQueen RB, Slejko JF. Toward Modified Impact Inventory Tables to Facilitate Patient-Centered Value Assessment. Pharmacoeconomics. 2021 Apr;39(4):379-382

Application to Rare Disease Therapies

- Effort to include heterogeneity across age, severity of disease, treatment options, ethnicity and race
  - Neuromyelitis optica spectrum disorder (NMOSD)
  - Spinal muscular atrophy (SMA)
  - Multiple myeloma (MM)
- NMOSD, SMA, and MM have recently approved and high priced therapies with potential for considerable benefit
- Focus groups ongoing with results projected by fall 2021

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Limitations

- Representativeness of selected participants to the whole disease group
  - Capturing diversity of patient voices attempted but difficult in rare disease
  - Initial focus groups in English; Spanish language materials developed in next iteration
- “Performance scores” for therapies on additional value criteria not measured leaves gap in MCDA applications
- Improvements on conventional value assessment methods still needed

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Summary and Implications

- "Modified impact inventory table" of value criteria
  - Tool for evidence generation
  - Input to augmented CEA, MCDA, and deliberations/voting discussions
- Input to MCDA exercises for pValue
  - MCDA scores a function of preferences by perspective
- MCDA applications
  - Direct weighting of thresholds
  - **Include scores as an additional consequence summarizing all additional criteria**

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Questions and contact information

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  - https://pharmacy.cuanschutz.edu/research/research-centers/pvalue

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