INNOVATION AND VALUE INITIATIVE

Novel Approaches to Identify and Quantify Patient Input for Health Technology Assessments (HTA)

ISPOR EU 2021

Speakers

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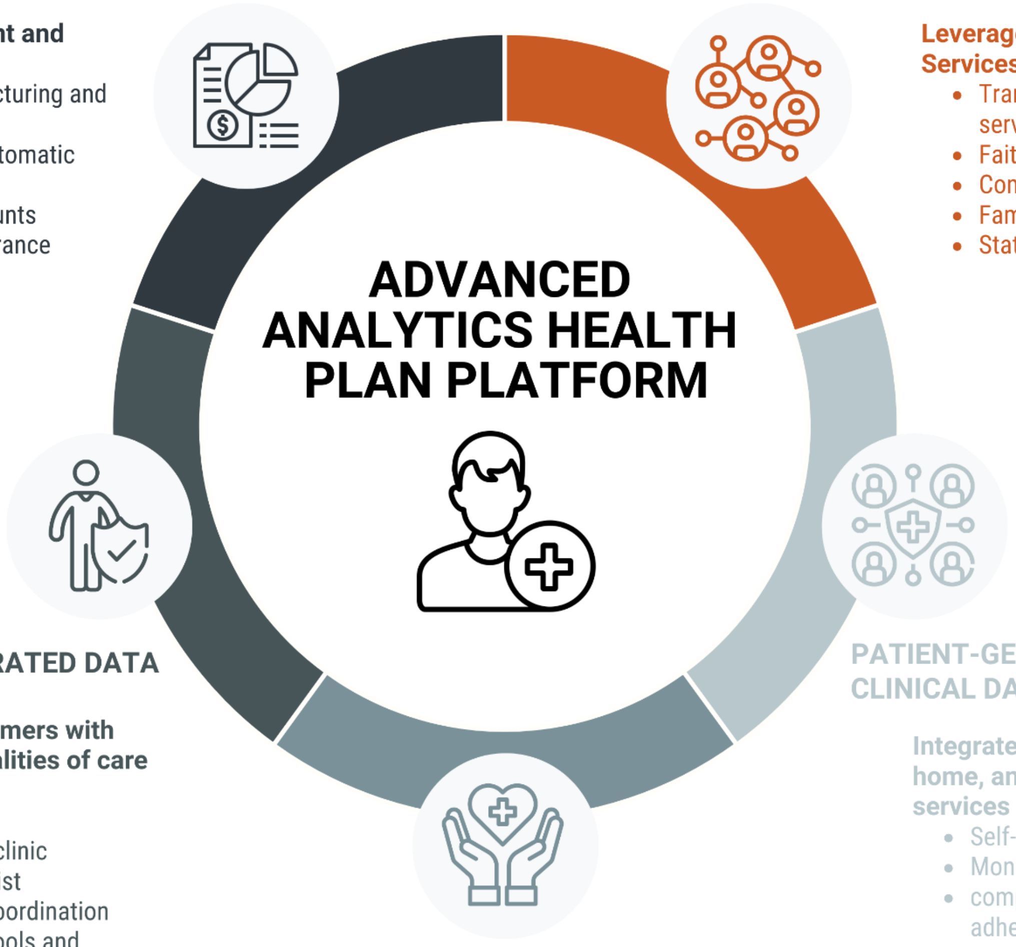
Introduction

- Economic models used to inform HTA usually fail to capture comprehensive clinical and economic outcomes of importance to patients or consider value elements that influence patient decision-making.
- Gaps in existing methods to systematically identify and quantify patient input for use in HTA.
- Various promising approaches have been proposed and are being tested.
- Objective: Demonstrate novel methods to systemically identify and quantify patient input, and briefly discuss importance and challenges of incorporating patient input in HTA.

Healthcare Ecosystems



ADVANCED ANALYTICS HEALTH PLAN PLATFORM

FINANCIAL DATA

Supports Payment and Financing
- Payment structuring and financing
- Digital and automatic payments
- Savings accounts
- Benefits/insurance coverage

SOCIAL STRUCTURE DATA

Leverages Support Services
- Transportation service
- Faith institutions
- Community
- Family
- State assistance

PROVIDER-GENERATED DATA

Connects consumers with traditional modalities of care
- Pharmacy
- Hospital
- Ambulatory clinic
- PCP/specialist
- Care team coordination
- Diagnostic tools and support
- Scheduling
- Quality

HEALTH AND WELLNESS DATA

Tracks daily life activities
- Nutrition
- Fitness
- PT and rehab

PATIENT-GENERATED CLINICAL DATA

Integrates home, near-home, and virtual care services
- Self-service solutions
- Monitoring tools
- compliance and adherence tools
- Home health
- Virtual care
- Retail clinics

Credit: McKinsey & Company. Source: https://www.mckinsey.com/industries/healthcare-systems-and-services/our-insights/the-next-wave-of-healthcare-innovation-the-evolution-of-ecosystems#

Framing the Problem

The goal: the right care, to the right patient, at the right time

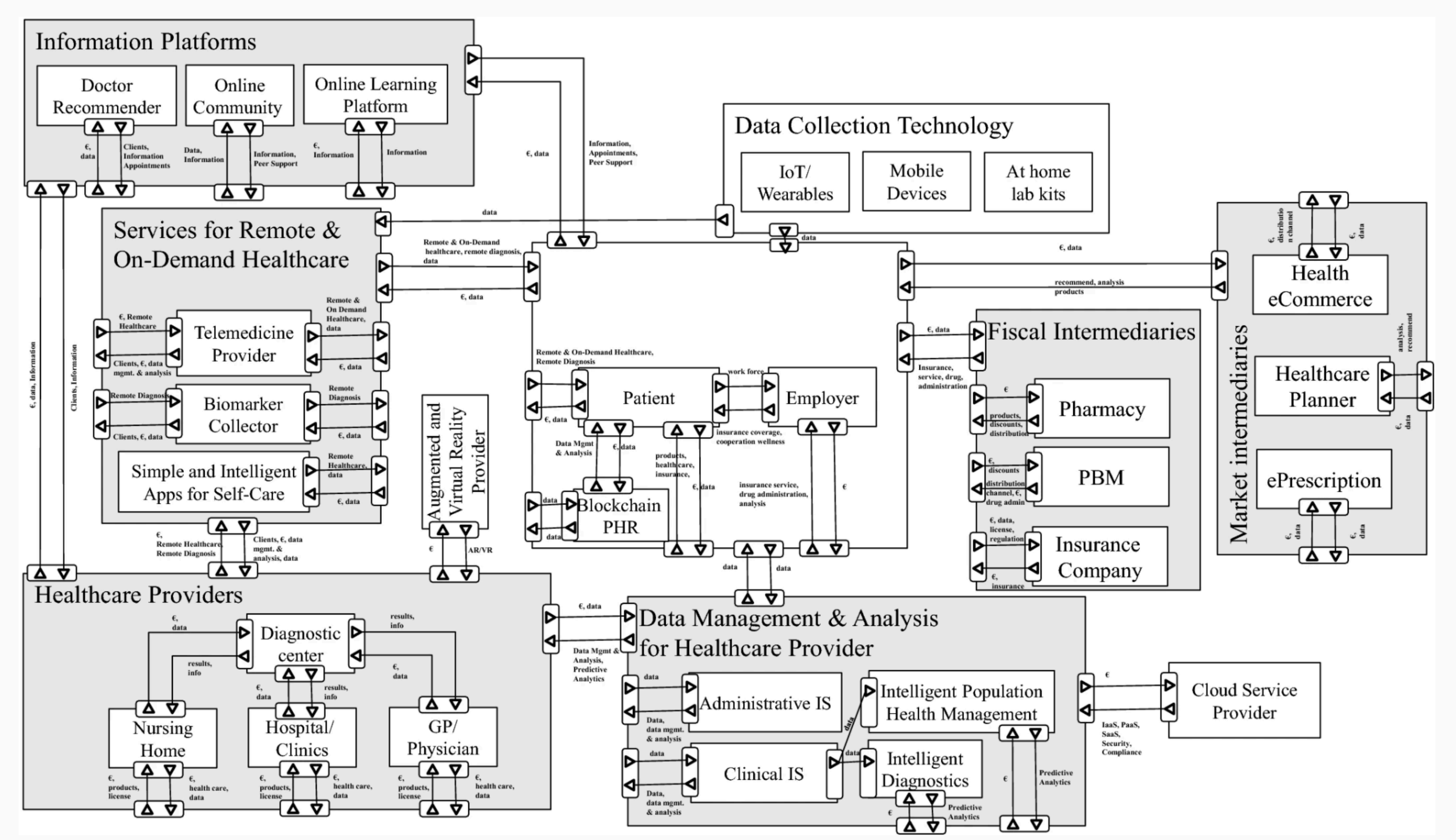
- Patients are unique and diverse in their histories, health goals, and experiences of care
- Patient, family, and caregiver experience is a vital - and often missing - component of measuring quality and value

Patient data is everywhere…and nowhere

- U.S. healthcare marketplace is flooded with data - but not representative, accessible, useable or useful
- Data landscape is siloed, from clinical trials to service delivery to patient-reported outcomes of care

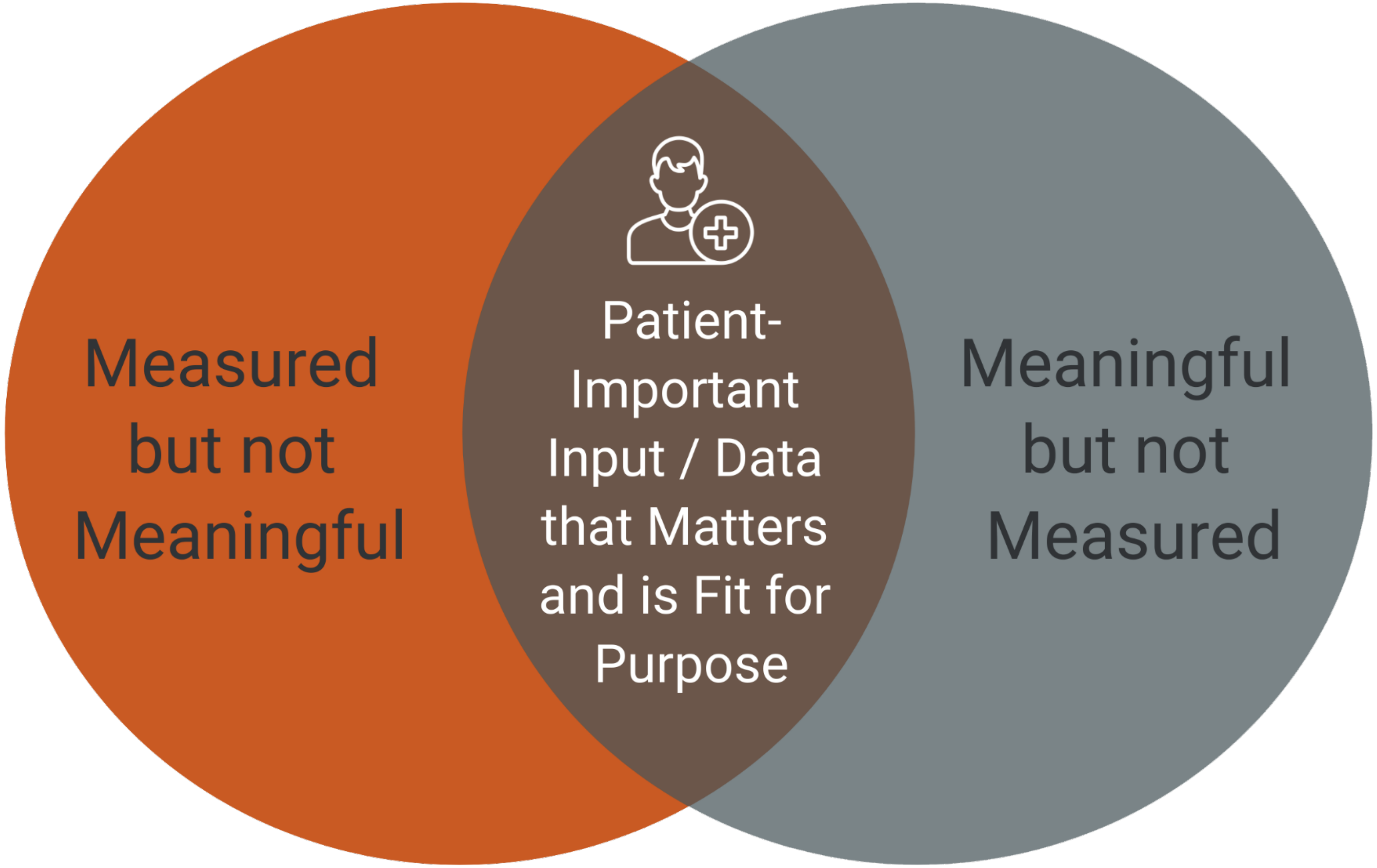
Data-driven decisions are imperative, but are disconnected from patients

- Decision-makers - at all levels - need access to effective tools to utilize patient data to drive decisions



From: Hermes, S., Riasanow, T., Clemons, E.K. et al. The digital transformation of the healthcare industry: exploring the rise of emerging platform ecosystems and their influence on the role of patients. Bus Res 13, 1033–1069 (2020). https://doi.org/10.1007/s40685-020-00125-x.

Prioritizing the Right Inputs to Drive Meaningful Insights



Measured but not Meaningful

Patient-Important Input / Data that Matters and is Fit for Purpose

Meaningful but not Measured

Polling Question 1

What are the biggest barriers to representing the patient voice in value assessment? (select the top 2)

- Availability of real-world data
- Methods used to capture patient input in value assessment
- Acceptance of patient experience data in value assessments
- Limited use of patient-reported outcome instruments in registration trials

Importance & Challenges of Incorporating Patient Input in HTA

- Drs. dosReis and Slejko will discuss using PAVE Center’s patient-informed value elements to elicit and quantify patient input on value elements in MDD treatment and translate this into meaningful patient-driven HTA.
- Dr. Frank will discuss using goal attainment scaling and work with patient communities to “crowd source” patient input in rheumatoid arthritis, which may be used to inform MCDA.
- Researchers will gain insight into available methods to incorporate patient input into methods to inform HTA.
- Innovators and patient groups will learn about how to ensure comprehensive patient input is incorporated into the data collection process.
- Payers and HTA bodies can learn about specific approaches useful for integrating patient input into decision-making.

Eliciting Patient Values in Major Depressive Disorder Treatment to Inform Translation into Patient-Driven HTA

Susan dosReis, PhD
Julia F. Slejko, PhD

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PAVE

PATIENT-DRIVEN
VALUES *in* HEALTHCARE
EVALUATION

Acknowledgments

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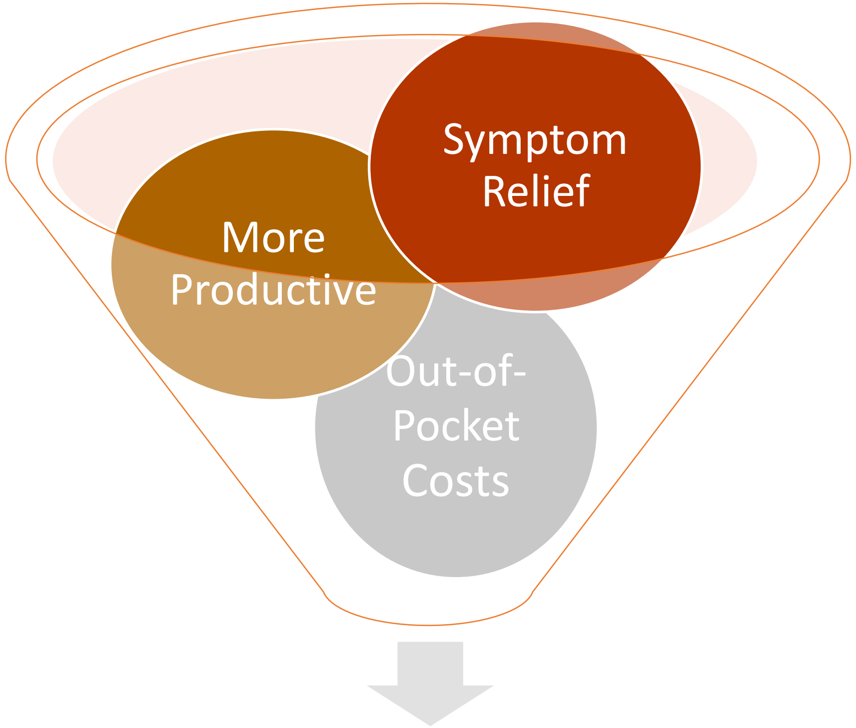
We are grateful to the patient communities and stakeholders who contributed their expertise, without which this work would not be possible

PAVE

Background



Preferences Reflect Patient Values

- Experiences with managing a health condition influence one’s treatment decisions
- Individuals develop preferences for specific attributes of treatment, including tolerance of benefits and risks
- The value of treatment is a balance of the relative importance of different treatment attributes

Patient preferences are a balance of the relative importance of treatment effects, outcomes, and costs.

PAVE

Background

- Several dilemmas in the application of a patient-driven health technology assessment (HTA)
  - Determining the elements of value that individuals ascribe to a treatment
  - Eliciting this information
  - Prioritizing value elements so that it may be translated for use in HTA

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]

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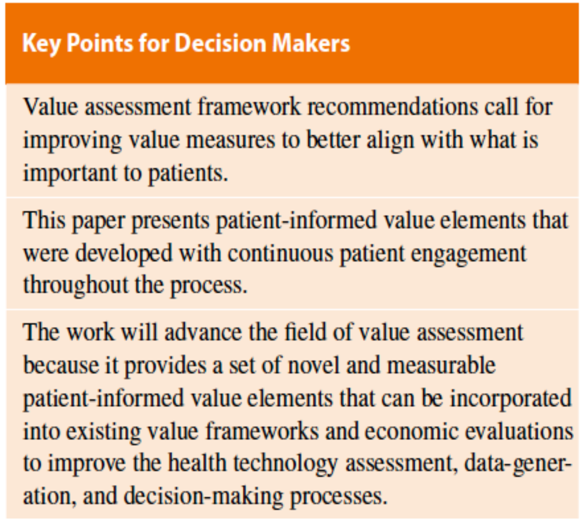
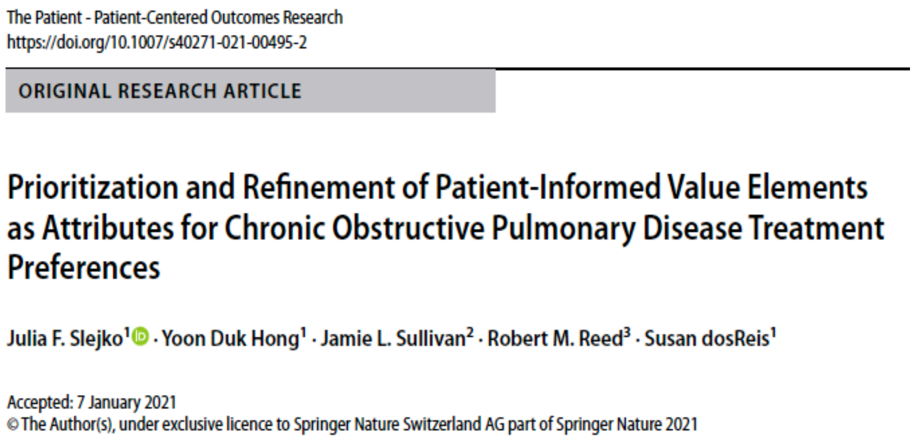


Key Points for Decision Makers

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.



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
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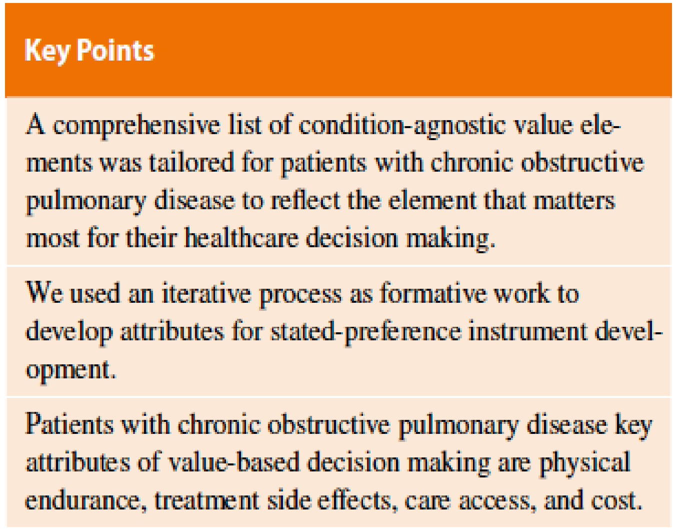


Key Points

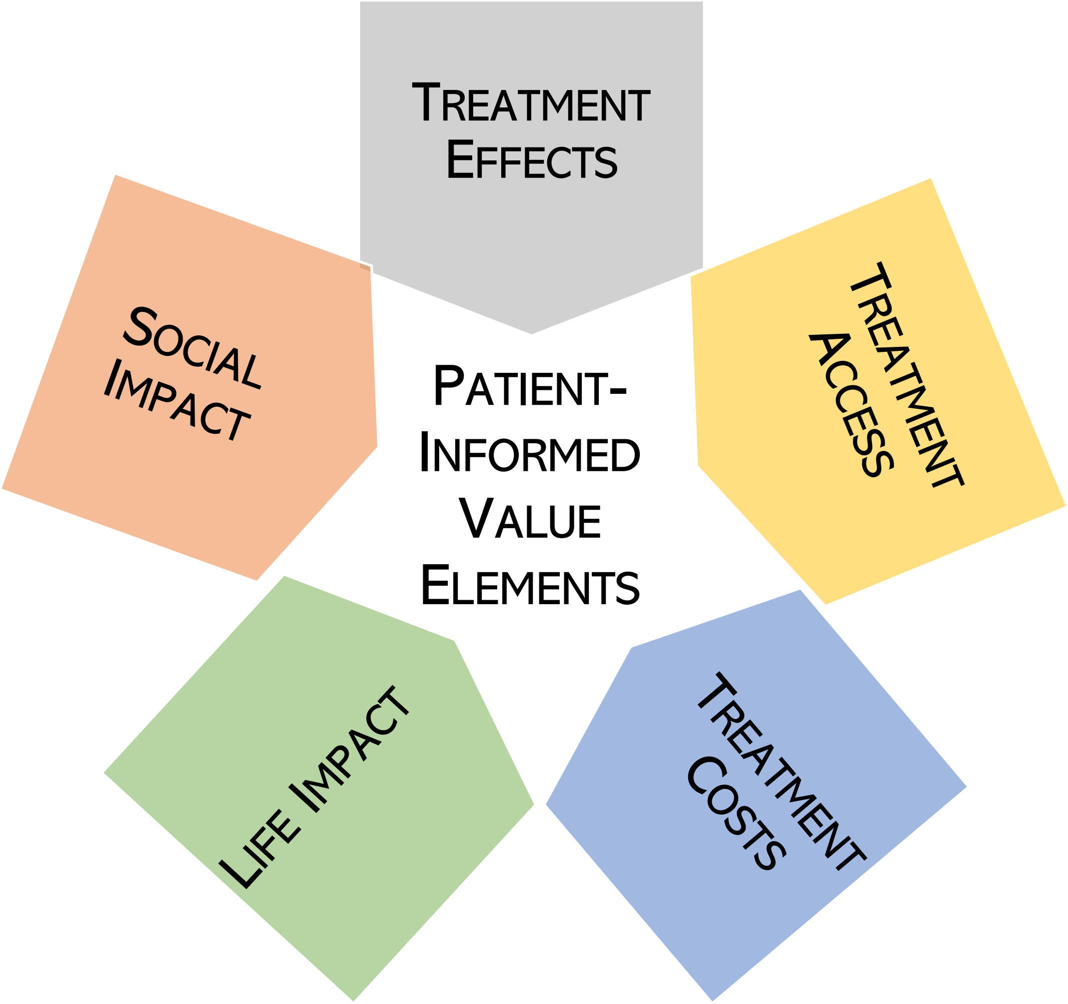
A comprehensive list of condition-agnostic value elements was tailored for patients with chronic obstructive pulmonary disease to reflect the element that matters most for their healthcare decision making.

We used an iterative process as formative work to develop attributes for stated-preference instrument development.

Patients with chronic obstructive pulmonary disease key attributes of value-based decision making are physical endurance, treatment side effects, care access, and cost.

Background

- PAVE’s patient-informed value elements
  - Domains:
    1. Treatment effects
    2. Treatment Access
    3. Cost
    4. Social impact
    5. Life impact
- Disease agnostic but can be tailored to specific conditions, treatments, and/or service delivery models/interventions


TREATMENT EFFECTS

SOCIAL IMPACT

PATIENT-INFORMED VALUE ELEMENTS

TREATMENT ACCESS

LIFE IMPACT

TREATMENT COSTS

Objectives

Three objectives to demonstrate the application of the patient-informed value elements:

(1) Elicit the value elements that influence treatment decisions

(2) Prioritize value elements for treatment of major depressive disorder (MDD)

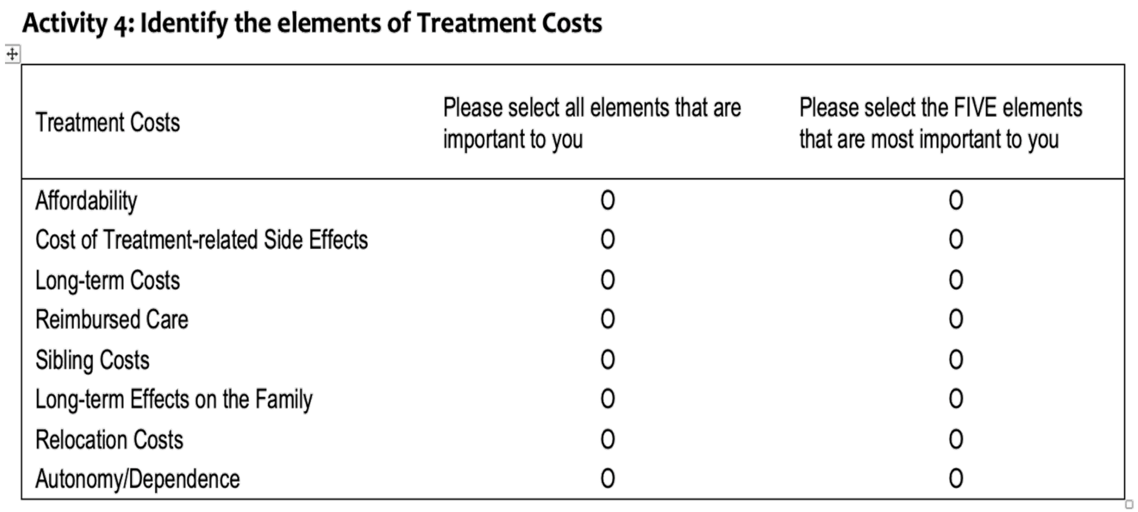
(3) Translate the value elements into a patient-driven HTA

Methodological Approach

- Multi-phase process with continual stakeholder engagement
  - Elicitation
    - Narrow to the most important value elements within each of the five domains
    - Contextualize the value element to an attribute of a treatment or intervention
    - Select key attributes and refine the language to mimic real-life decision-making
  - Quantification
    - Use a stated preference tool to collect information on preferences
    - Analyze the data and generate preference utility scores
  - Translation
    - Incorporate the preference utility into an economic evaluation

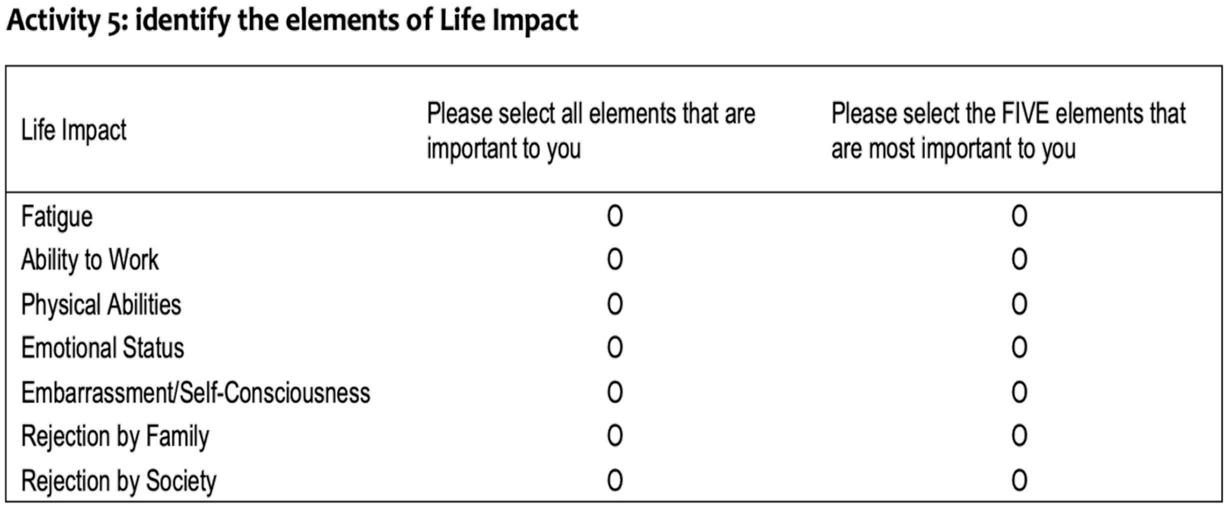
Elicitation of Value Elements

- Qualitative research
  - Individual interviews obtain personal experiences in their own words
  - Guided activities elicit important value elements



Activity 4: Identify the elements of Treatment Costs

Treatment Costs	Please select all elements that are important to you	Please select the FIVE elements that are most important to you
Affordability	○	○
Cost of Treatment-related Side Effects	○	○
Long-term Costs	○	○
Reimbursed Care	○	○
Sibling Costs	○	○
Long-term Effects on the Family	○	○
Relocation Costs	○	○
Autonomy/Dependence	○	○



Activity 5: identify the elements of Life Impact

Life Impact	Please select all elements that are important to you	Please select the FIVE elements that are most important to you
Fatigue	○	○
Ability to Work	○	○
Physical Abilities	○	○
Emotional Status	○	○
Embarrassment/Self-Consciousness	○	○
Rejection by Family	○	○
Rejection by Society	○	○

Prioritization of Value Elements

Participant-selected Elements of Most Importance

↓

Patient Stakeholder Advisory Group Narrows to Key Elements

↓

Derive Candidate Attributes from the Key Elements

↓

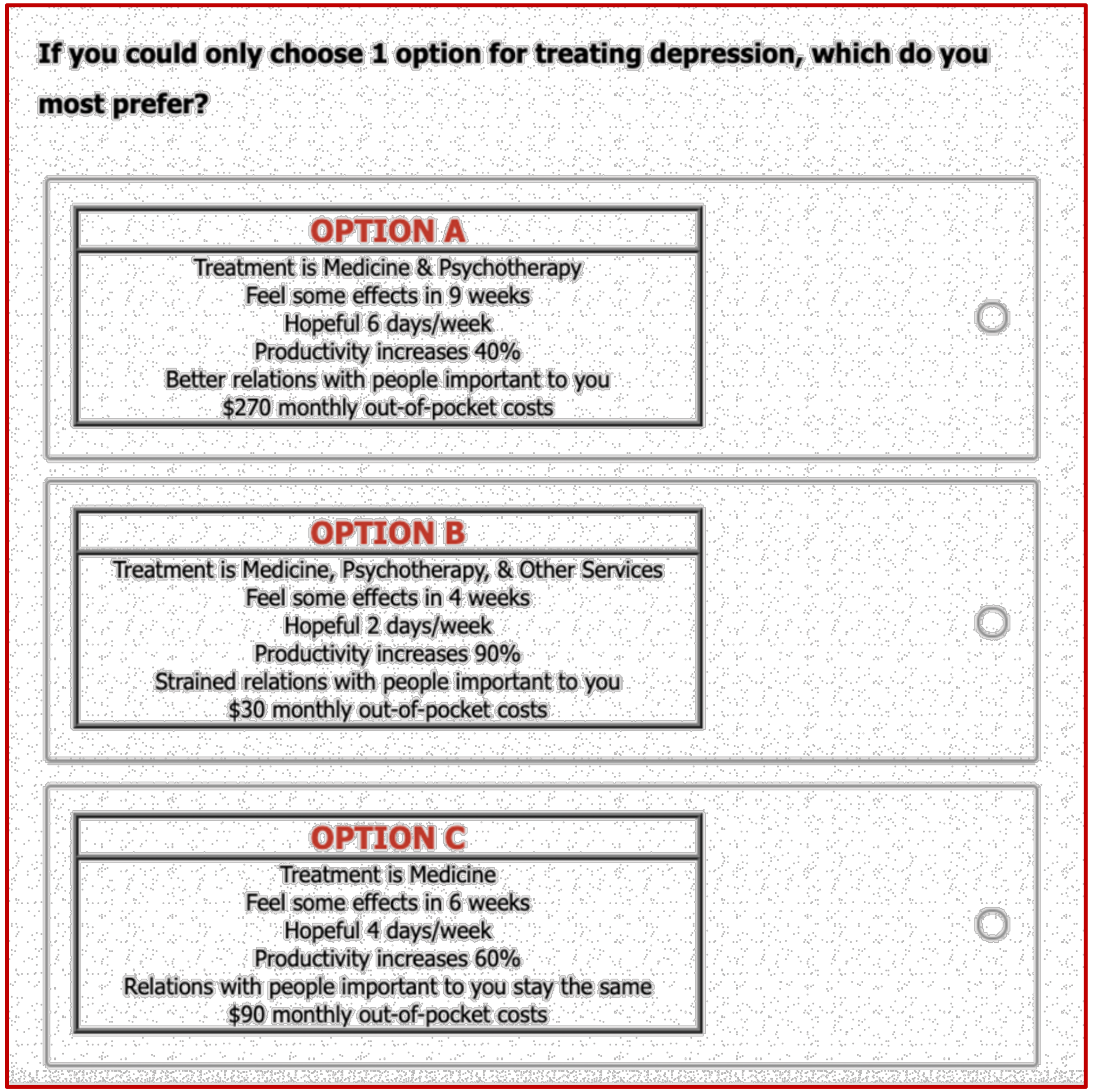
Refine Candidate Attributes

↓

Develop a Patient Preference Measurement Tool

Preference Measurement

- Stated Preferences methods to quantify:
  - Relative importance of treatment attributes
  - Threshold for trade-offs between benefits/risks and costs
  - Difference in treatment preferences across population segments



If you could only choose 1 option for treating depression, which do you most prefer?

OPTION A
Treatment is Medicine & Psychotherapy
Feel some effects in 9 weeks
Hopeful 6 days/week
Productivity increases 40%
Better relations with people important to you
$270 monthly out-of-pocket costs
○

OPTION B
Treatment is Medicine, Psychotherapy, & Other Services
Feel some effects in 4 weeks
Hopeful 2 days/week
Productivity increases 90%
Strained relations with people important to you
$30 monthly out-of-pocket costs
○

OPTION C
Treatment is Medicine
Feel some effects in 6 weeks
Hopeful 4 days/week
Productivity increases 60%
Relations with people important to you stay the same
$90 monthly out-of-pocket costs
○

Gaps in existing VA for MDD

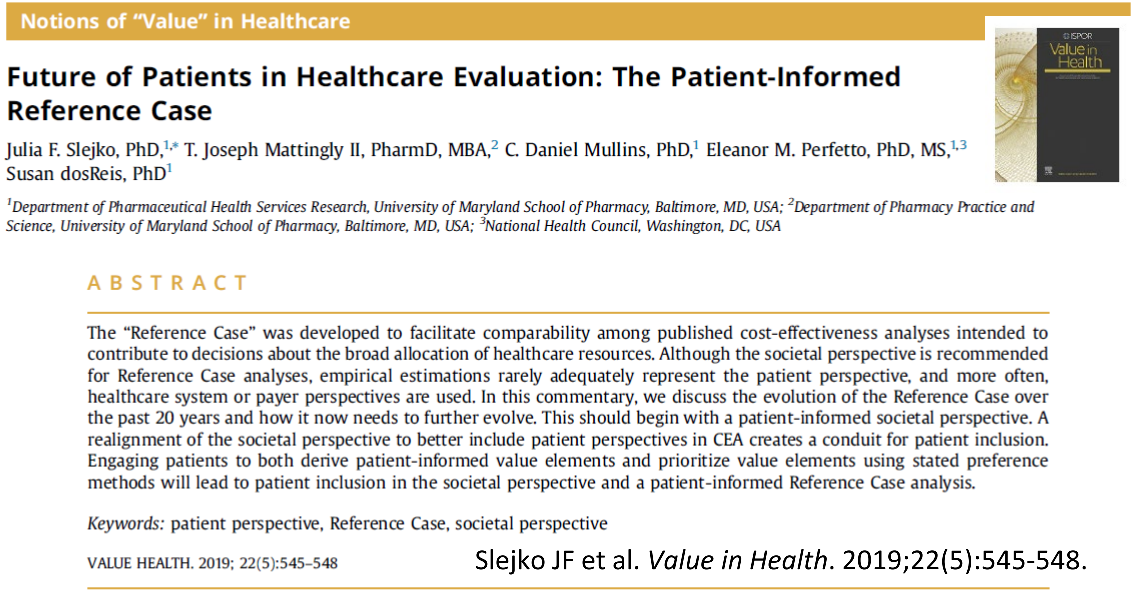
- Do previous CEA models address the elements found to be important to patients?

Mode of Treatment	**Time to Treatment Helpfulness**	**MDD Symptom Relief**
Quality of Work	**Interaction with Others**	**Affordability**

- 83 published MDD studies were reviewed
  - Mode of treatment, effects on symptoms, and time to effect were often key parameters.
  - Productivity was included as a model parameter in 35 studies (42%).
  - Five studies (6%) included out-of-pocket costs.
  - Interaction with others was difficult to ascertain, potentially covered by HRQoL.

Model Perspective for MDD

- A model built from the payer or healthcare system perspective is unlikely to have components that are patient-centered
    - The societal perspective may include inputs important to patients.
- 43% of studies used a societal perspective for their analysis
    - Several studies reported some patient-specific measures within this perspective.



Notions of "Value" in Healthcare

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

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ABSTRACT

The "Reference Case" was developed to facilitate comparability among published cost-effectiveness analyses intended to contribute to decisions about the broad allocation of healthcare resources. Although the societal perspective is recommended for Reference Case analyses, empirical estimations rarely adequately represent the patient perspective, and more often, healthcare system or payer perspectives are used. In this commentary, we discuss the evolution of the Reference Case over the past 20 years and how it now needs to further evolve. This should begin with a patient-informed societal perspective. A realignment of the societal perspective to better include patient perspectives in CEA creates a conduit for patient inclusion. Engaging patients to both derive patient-informed value elements and prioritize value elements using stated preference methods will lead to patient inclusion in the societal perspective and a patient-informed Reference Case analysis.

Keywords: patient perspective, Reference Case, societal perspective

VALUE HEALTH. 2019; 22(5):545–548

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

Translate MDD Findings to VA

Implementing the Patient-Informed Reference Case

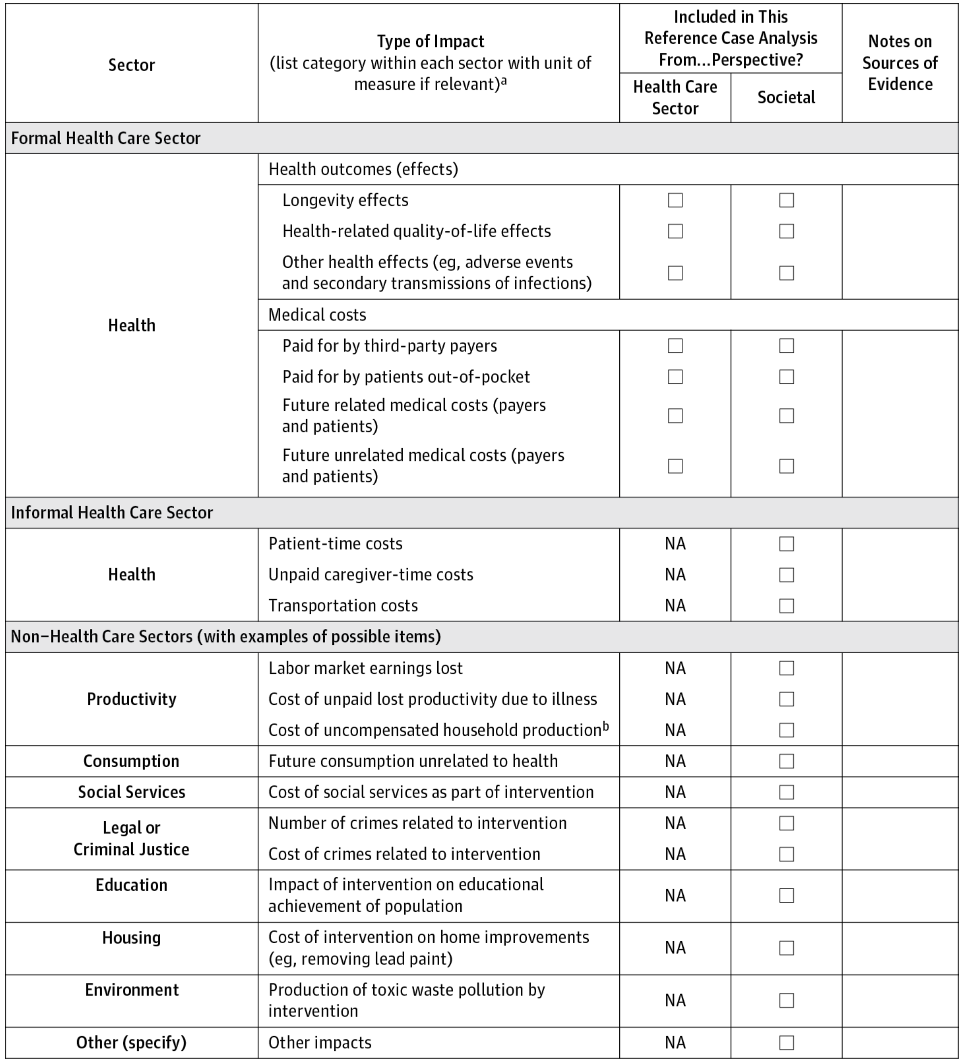
Health States and Outcomes:	Reflect patient-important outcomes using measures that are meaningful to patients (e.g., days of feeling hopeful)
Patient Engagement in VA:	Inform uncertainty in model structure or inputs Allow model structure to reflect a range of treatment types in comparisons Enable parameter uncertainty to reflect the importance of individual attributes (e.g., data on time to treatment helpfulness)
Preference Heterogeneity:	Preference subtypes based on patients' willingness to trade off treatment attributes for out-of-pocket costs; subgroup analysis
CEA Outputs:	Productivity was a key attribute important to patients and could be reflected as an alternative denominator

Polling Question 2

Of the four categories for translation into value assessments, which appears most feasible, in your opinion:

- Patient engagement in VA
- Preference heterogeneity
- Health states and outcomes
- CEA outputs

Leveraging Existing Tools



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	☐	☐	
	Health-related quality-of-life effects	☐	☐	
	Other health effects (eg, adverse events and secondary transmissions of infections)	☐	☐	
	Medical costs			
	Paid for by third-party payers	☐	☐	
	Paid for by patients out-of-pocket	☐	☐	
	Future related medical costs (payers and patients)	☐	☐	
	Future unrelated medical costs (payers and patients)	☐	☐	
Informal Health Care Sector				
Health	Patient-time costs	NA	☐	
	Unpaid caregiver-time costs	NA	☐	
	Transportation costs	NA	☐	
Non–Health Care Sectors (with examples of possible items)				
Productivity	Labor market earnings lost	NA	☐	
	Cost of unpaid lost productivity due to illness	NA	☐	
	Cost of uncompensated household production[b]	NA	☐	
Consumption	Future consumption unrelated to health	NA	☐	
Social Services	Cost of social services as part of intervention	NA	☐	
Legal or Criminal Justice	Number of crimes related to intervention	NA	☐	
	Cost of crimes related to intervention	NA	☐	
Education	Impact of intervention on educational achievement of population	NA	☐	
Housing	Cost of intervention on home improvements (eg, removing lead paint)	NA	☐	
Environment	Production of toxic waste pollution by intervention	NA	☐	
Other (specify)	Other impacts	NA	☐	

JAMA. 2016;316(10):1093-1103. doi:10.1001/jama.2016.12195

PharmacoEconomics
https://doi.org/10.1007/s40273-021-01017-6

EDITORIAL

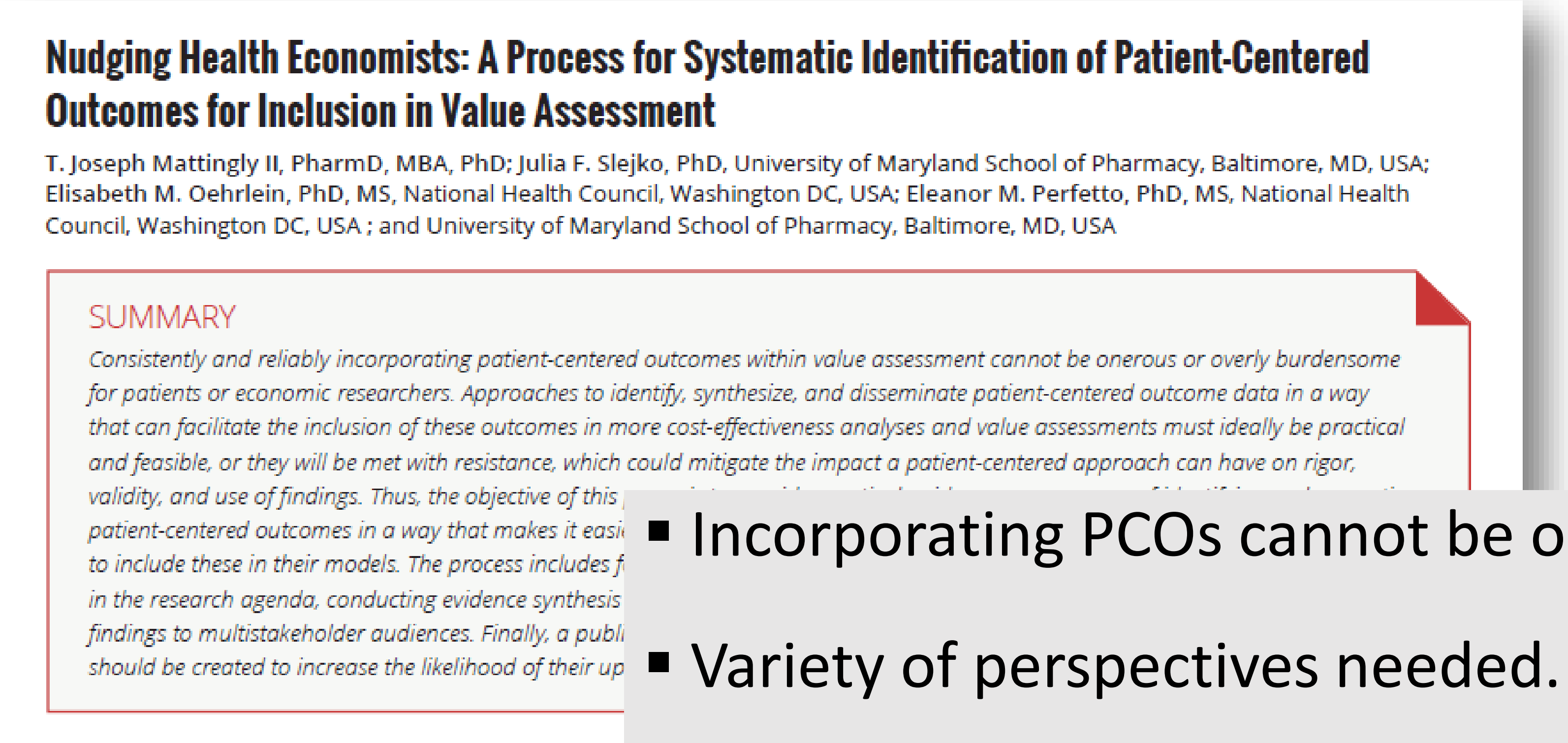
Toward Modified Impact Inventory Tables to Facilitate Patient-Centered Value Assessment

R. Brett McQueen[1,2] · Julia F. Slejko[1,2]

Accepted: 8 March 2021

- Modify the impact inventory table for specific conditions.
- Existing domains may already comprise these elements (depends on perspective).
- Expand domains as needed.

Recommended Practices



Nudging Health Economists: A Process for Systematic Identification of Patient-Centered Outcomes for Inclusion in Value Assessment

T. Joseph Mattingly II, PharmD, MBA, PhD; Julia F. Slejko, PhD, University of Maryland School of Pharmacy, Baltimore, MD, USA; Elisabeth M. Oehrlein, PhD, MS, National Health Council, Washington DC, USA; Eleanor M. Perfetto, PhD, MS, National Health Council, Washington DC, USA ; and University of Maryland School of Pharmacy, Baltimore, MD, USA

SUMMARY

Consistently and reliably incorporating patient-centered outcomes within value assessment cannot be onerous or overly burdensome for patients or economic researchers. Approaches to identify, synthesize, and disseminate patient-centered outcome data in a way that can facilitate the inclusion of these outcomes in more cost-effectiveness analyses and value assessments must ideally be practical and feasible, or they will be met with resistance, which could mitigate the impact a patient-centered approach can have on rigor,
validity, and use of findings. Thus, the objective of this
patient-centered outcomes in a way that makes it easi
to include these in their models. The process includes f
in the research agenda, conducting evidence synthesis
findings to multistakeholder audiences. Finally, a publi
should be created to increase the likelihood of their up

- Incorporating PCOs cannot be onerous or burdensome.
- Variety of perspectives needed.
- VA is multidisciplinary – expertise in qualitative interviews/focus groups is needed.

Value & Outcomes Spotlight, September 2021

Thank you.

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Patient-Engaged Healthcare Valuation: Proof of Concept

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Piksel via GettyImages

Patient engagement in HTA – Why?

How could including patient communities improve HTA?

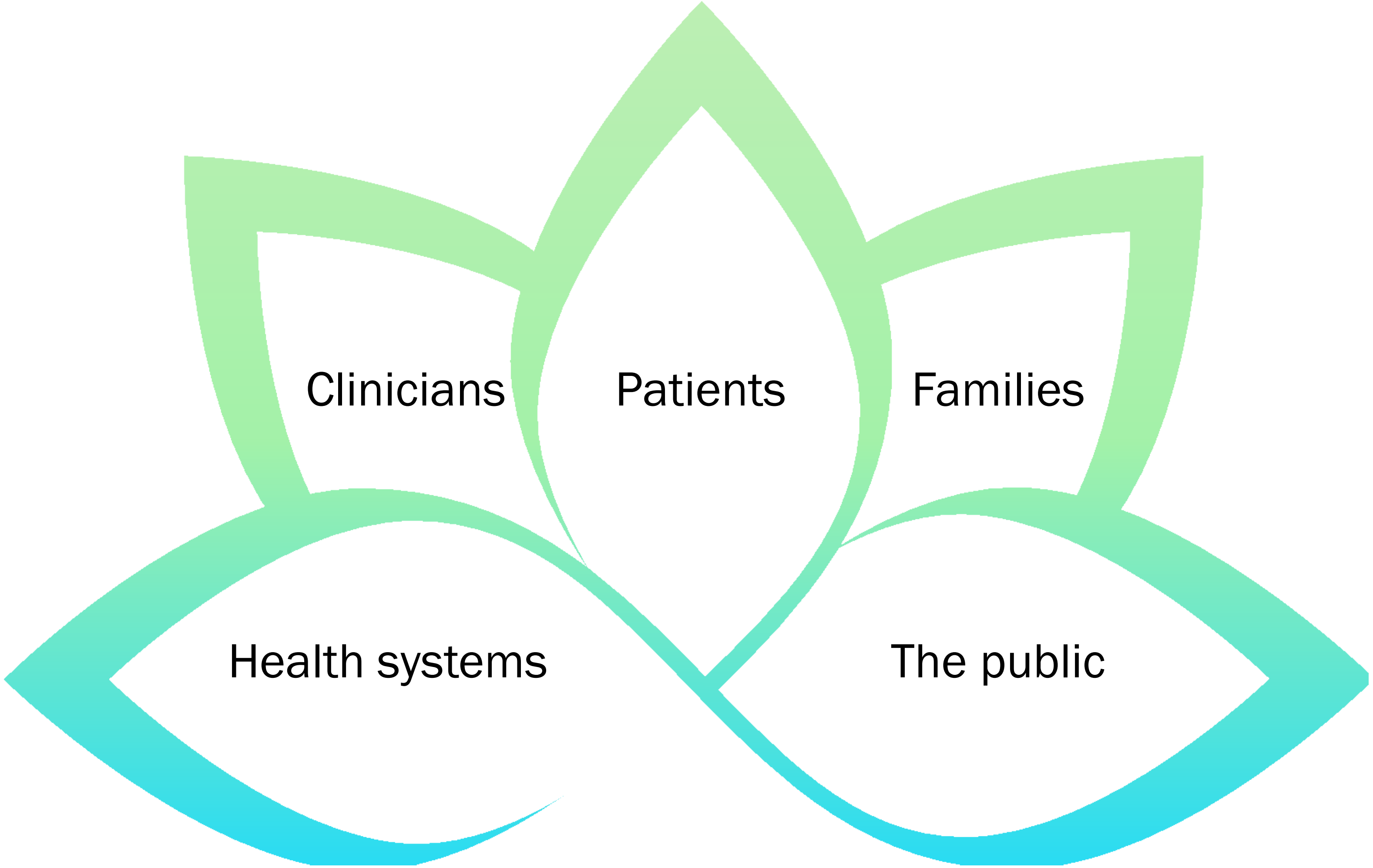
What methods would support engagement of patient communities?

Pros and Cons

Capturing inputs

Patients | Other Stakeholders |Public

Whose views inform HTA?



Clinicians

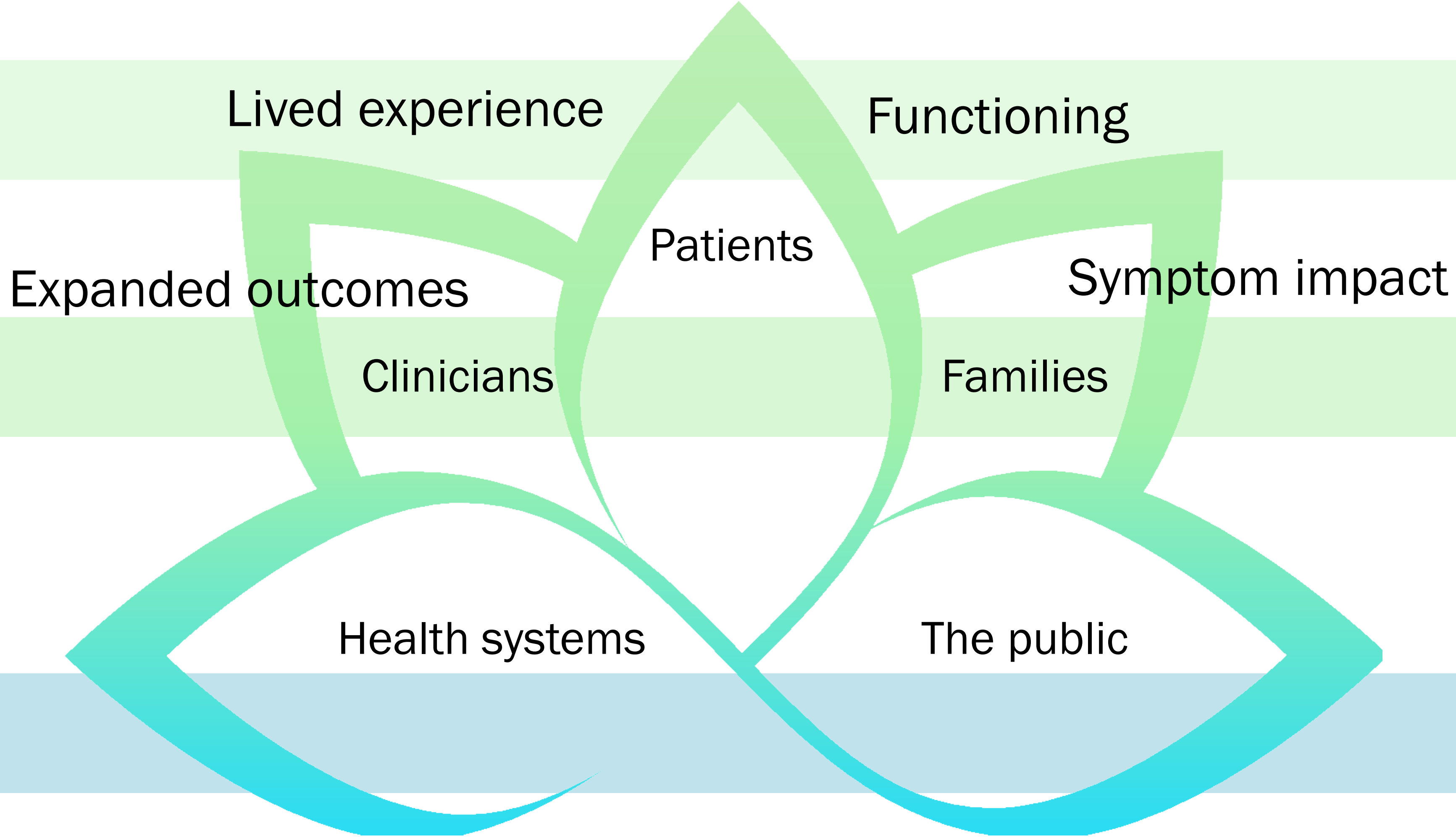
Patients

Families

Health systems

The public

HTA
Fully realized



Lived experience

Functioning

Patients

Expanded outcomes

Symptom impact

Clinicians

Families

Health systems

The public

Methods

1) Input from the project Steering Committee and review of literature

2) Drafting of goal items for pilot survey, cognitive interviews

3) Pilot survey and item refinement

4) Second round survey data collection

5) Final Steering Committee data review

Feasibility Pilot

Respondents

N=47

Women: 93%

White: 87%

College-educated: 72%

Age range: 28-72 years

Survey Domains

My goals for living with RA are to…

- Symptom Management – 8 items
- Life Impact – 7 items
- Managing My RA – 4 items
- Treatment Features – 12 items
- Other Goals

Item Importance Ratings

Not important

Somewhat important

Important

Very important

Importance Ratings

Symptom Management	Manage RA pain	🟩
	Improve the quality of my life with RA	🟩
	Reduce how my RA pain interferes with my life	🟩
	Reduce my tiredness or fatigue	🟨
	Limit surprises in how my RA symptoms affect me	🟨
	Improve my ability to do things like dress, eat, or walk	🟨
	Improve the quality of my sleep	🟧
	Reduce my morning stiffness	🟧
Life Impact	Be independent	🟩
	Do things that I enjoy doing	🟩
	Reduce life interference	🟩
	Reduce the ways that RA interferes with my ability to work/take care of my life	🟩
	Reduce symptom impact	🟨
	Be a friend/loved one despite symptoms	🟨
	Reduce social time interference	🟨
Managing my RA	Feel like I can manage my RA	🟩
	Reduce my worry or anxiety about my RA	🟨
	Reduce my sadness about my RA	🟧
	Feel more accepting of my RA	🟧
Treatment Features	Find treatments that are effective	🟩
	Have the information I need to make treatment decisions	🟩
	Know what to expect with treatment	🟩
	Reduce treatment impact on my life	🟩
	Understand treatment options	🟩
	Find treatments that have very few side effects	🟨
	Manageable side effects	🟨
	Find treatments that don’t cost too much	🟧
	Reduce treatment time	🟧

Sources of input

Clinicians

General public

Policymakers

More clinicians

Ideal sources of input

Clinicians

General public

Policymakers

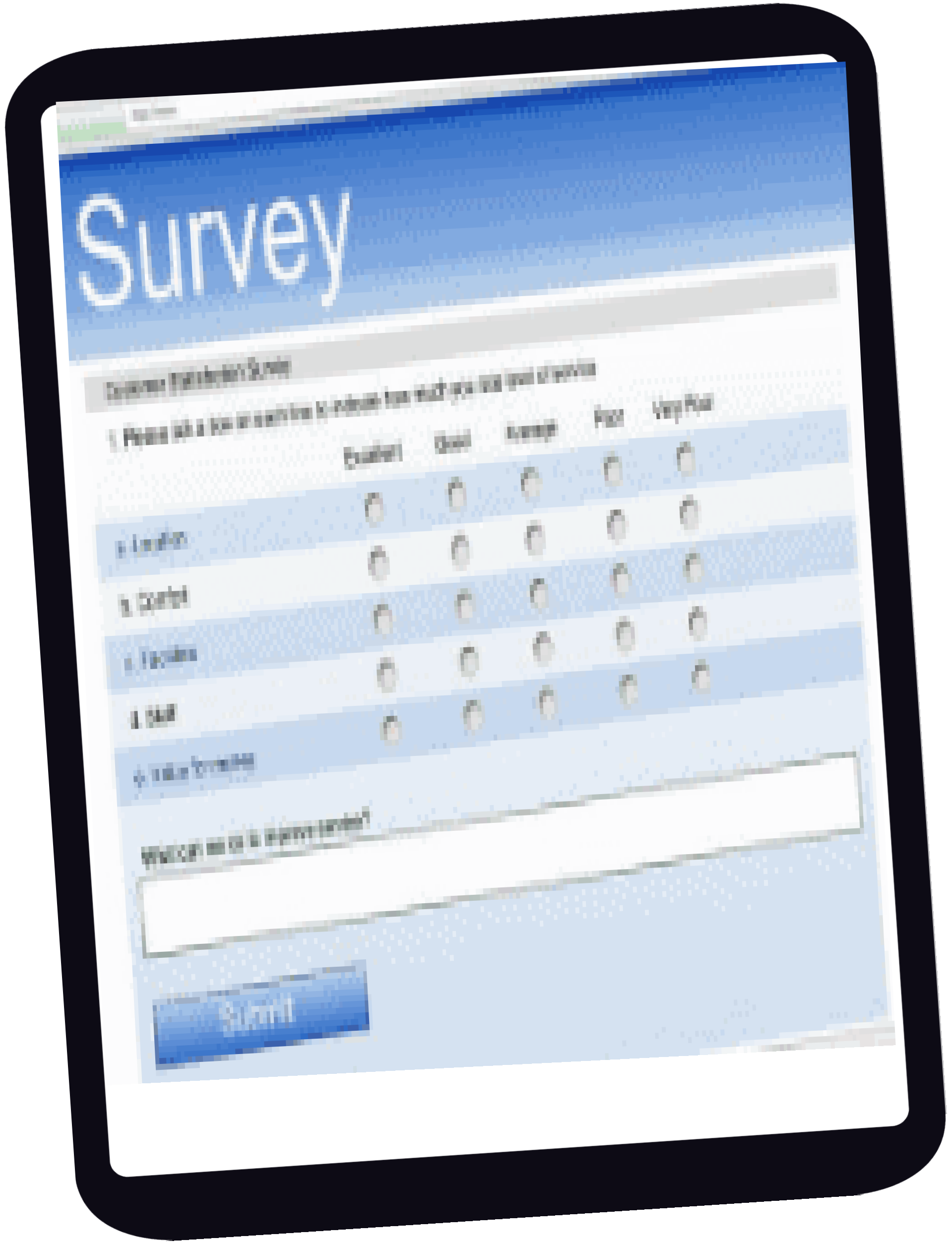
Patients

More clinicians

Goal Attainment Scaling

	Health Authorities	Health systems	Clinicians	Patients	The public
Identify Goal Sets	X	X	X	X	X
Collect Input				X	X
Deliberate	X	X	X	X	X

Feasibility



Improving feasibility

1. Pursue continued open-ended goal elicitation to ensure the goal set continues to reflect the patient community goals accurately.
2. Periodically review goals via patient/clinician GAS discussions to further enhance the fidelity of the goal set to the patient community of interest.

Scanrail via Getty Images

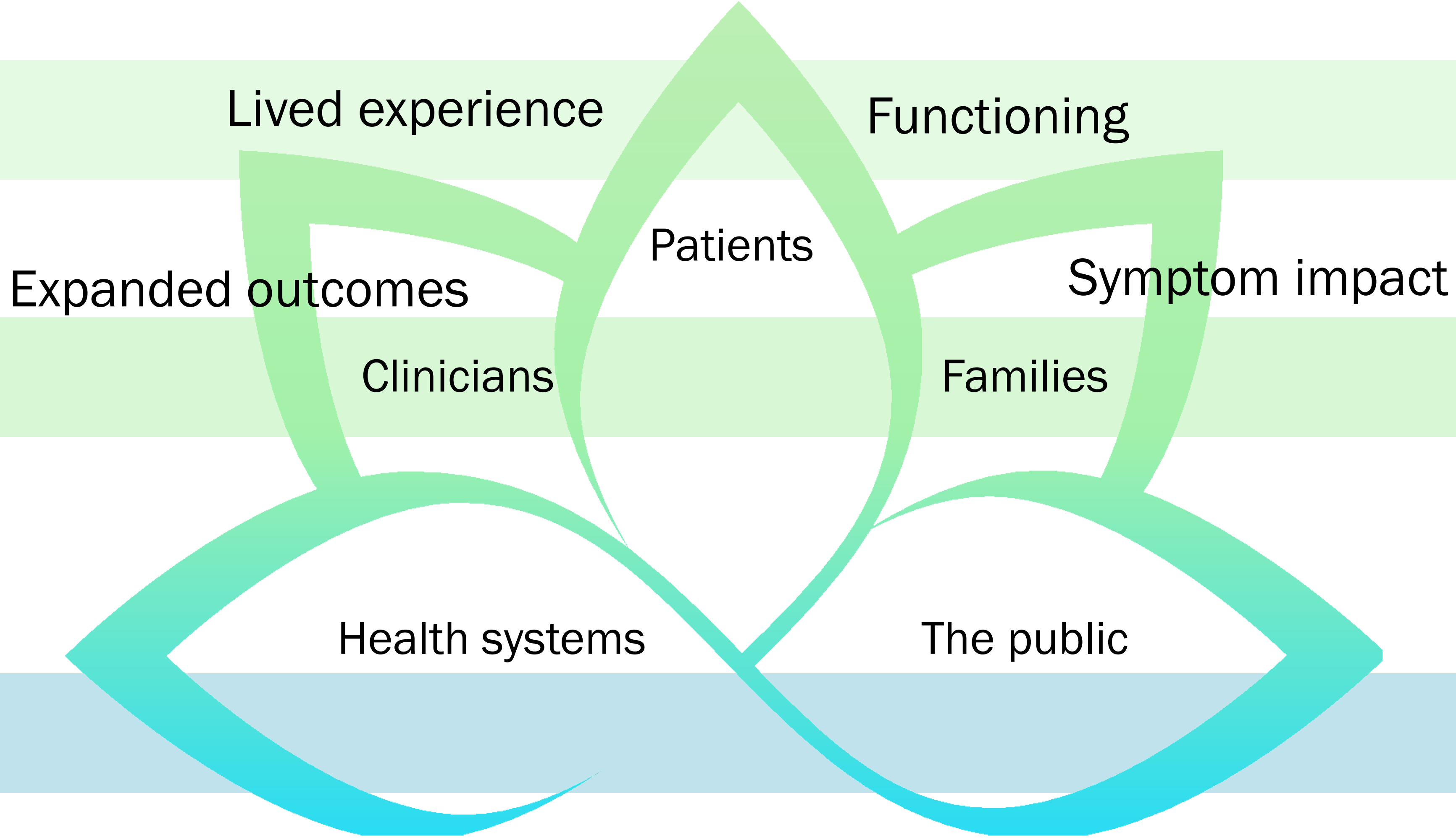
Conclusions

- Patients can identify and prioritize goals relevant for treatment evaluation and comparison.
- Patient-important goals collected using this method reflect the wide range of input from patients with lived experience and can be subsequently incorporated into deliverable healthcare valuation.

Next Steps

- Proactively address limitations of existing online patient networks in terms of gender, age, and race and ethnicity.
- Involve methodologists to aid with translating patient goals into MCDA weights.

HTA
Fully realized



Lived experience

Functioning

Patients

Expanded outcomes

Symptom impact

Clinicians

Families

Health systems

The public

RAND
CORPORATION

Polling Question 3

- Do you expect that incorporation of patient input will increase in Value Assessment/HTA over the next 3 years?

- Yes, I expect to see increased incorporation of patient input.
- No, I expect that things will stay at the current level.
- Unsure

Discussion