

What CAN be Gained from Payer/HTA Involvement in Multi-Stakeholder Development of Core Outcome Sets?

Intro by D. Messner, PhD, Center for Med Tech Policy

Virtual ISPOR 2021




COI Statement

- CMTP is an independent non-profit 501(c)(3)
- coreASTHMA and coreSCD were sponsored by precompetitive consortia of companies developing therapies for these conditions:
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- I do not personally consult for, nor have taken direct payments from, any of these companies and have no personal disclosures to make.



What are Core Outcome Sets*



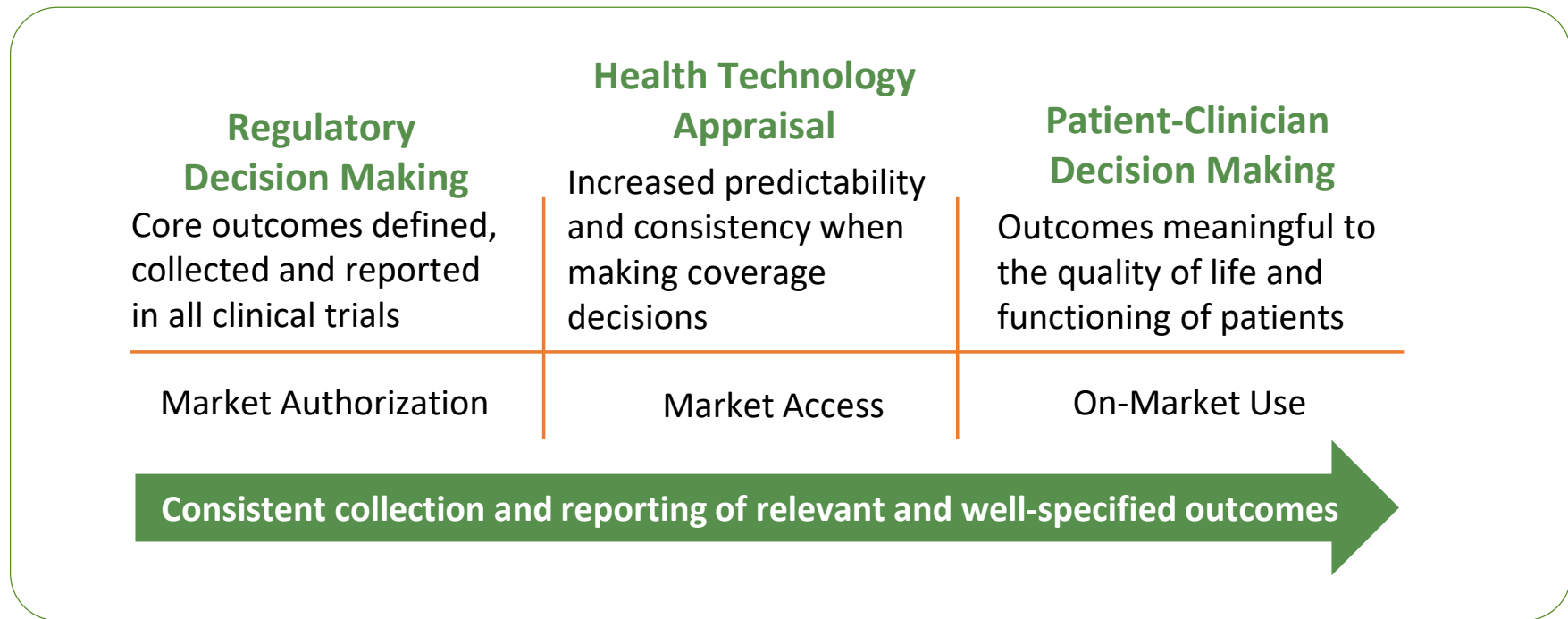
“An agreed standardised set of outcomes that should be measured and reported, as a minimum, in all clinical research in specific areas of health or health care”

(Our particular focus is usually late phase interventional trials, but COS applicable across clinical research contexts)

*Definition from the COMET (Core Outcome Measures in Effectiveness Trials) Initiative

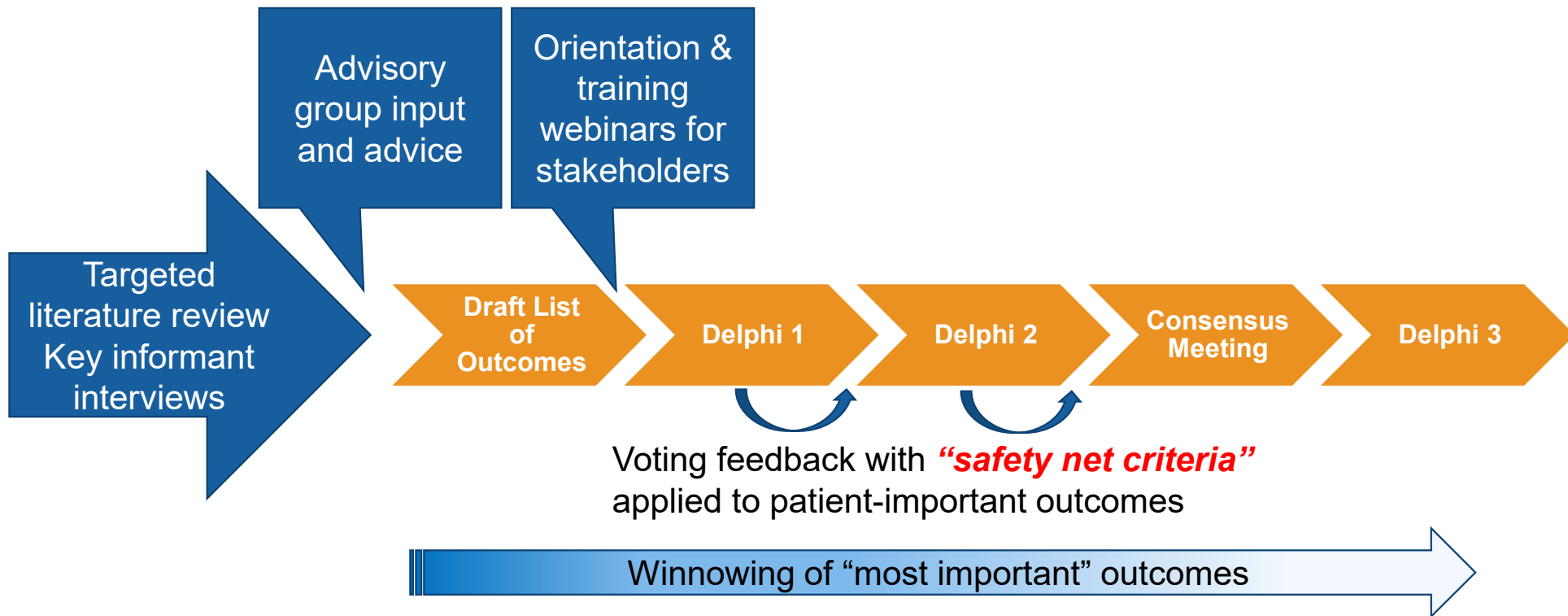
Why Do COS?

Consistent, Relevant Evidence

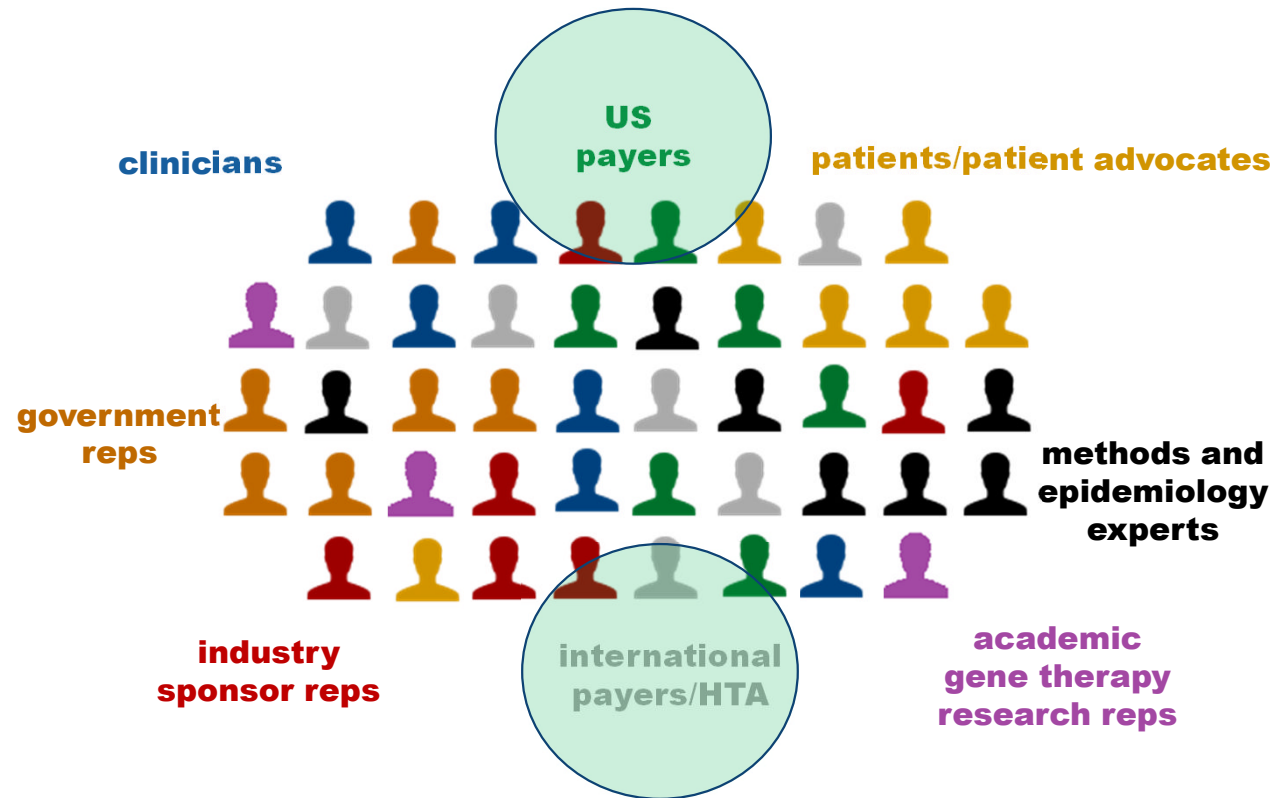


Skinner, Clearfield, Iorio, Tunis. Comparing Outcomes Across Clinical Trials: Core Outcome Set for Hemophilia Gene Therapy as a Model for Other Diseases (2017). DIA Global Forum Vol. 9 Issue 5

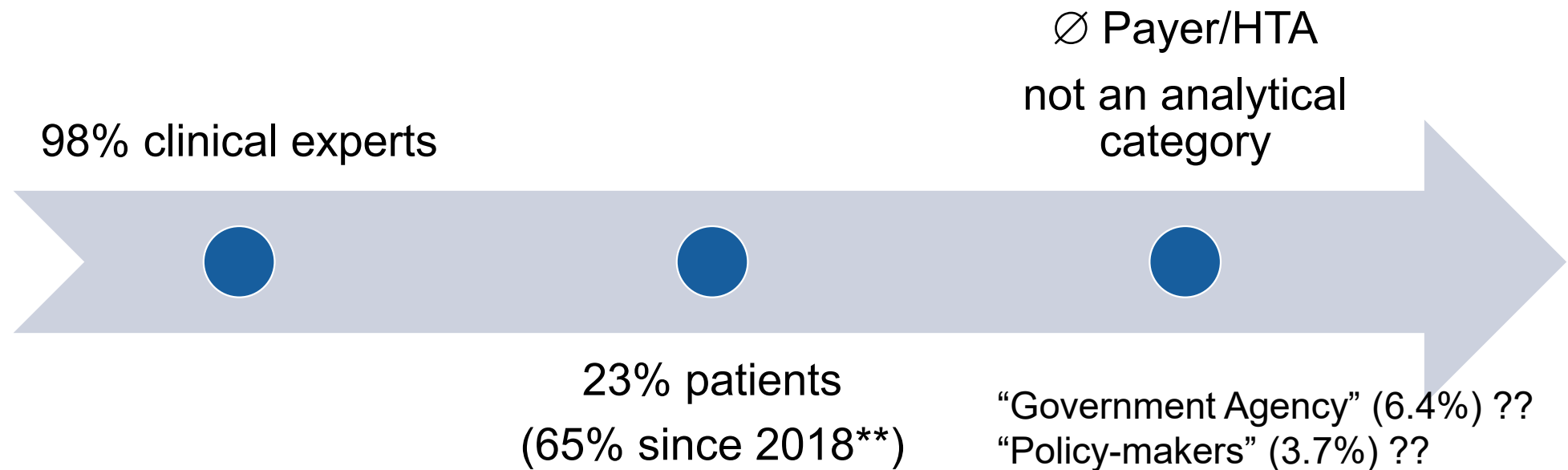
How do we do COS? Our approach



CMTP's Typical COS Stakeholders



Participants in COS since 1983*



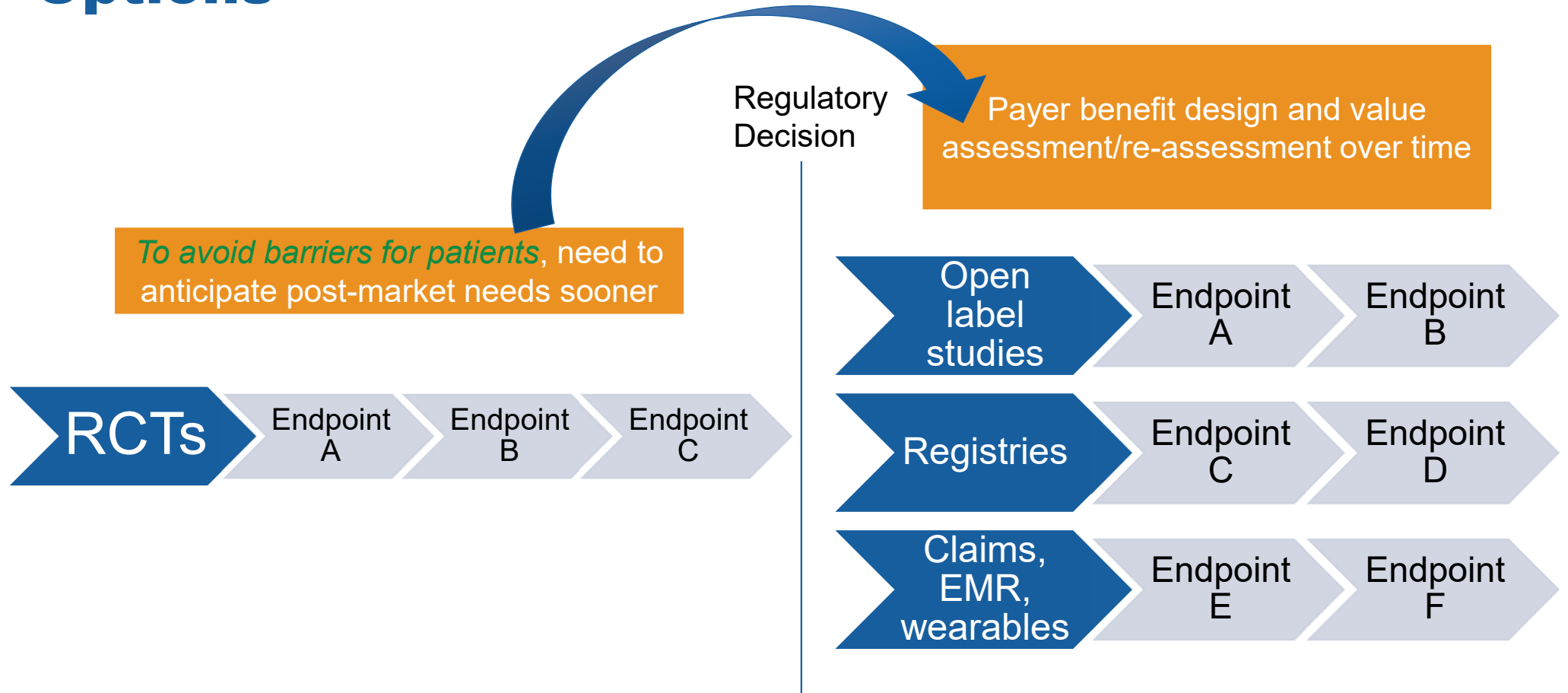
* Systematic review of COS (N = 294 studies), Gargon et al., PLOS ONE, Dec 28, 2018

**Biggane et al. Trials, [2018] 19; 113

If most groups haven't done so, why include HTA/payers?



Payer/HTA Decisions Affect Patient Care Options



Initially limited info for HTA & payer can be alleviated with premarket planning, including informed outcome selection

In the process... shared knowledge

Patient understanding of HTA

- e.g., measuring patient fatigue or chest “tightness”
- Reduction of resource use as reflection of treatment effectiveness

Payer/HTA (and others') understanding of Patient Priorities

- Reduction of corticosteroid use in asthma
- Neurocognitive function in sickle cell

Resonance between patient values and payer needs

- Common focus on “hard” clinical endpoints, improvements in patient function

Panel – Recent COS Participants

Thought Questions

- What were you able to bring to the project?
- What were you able to take away?
- Should more payers/HTA participate in COS development,
- What is potential impact of COS for future evidence-based policy-making?

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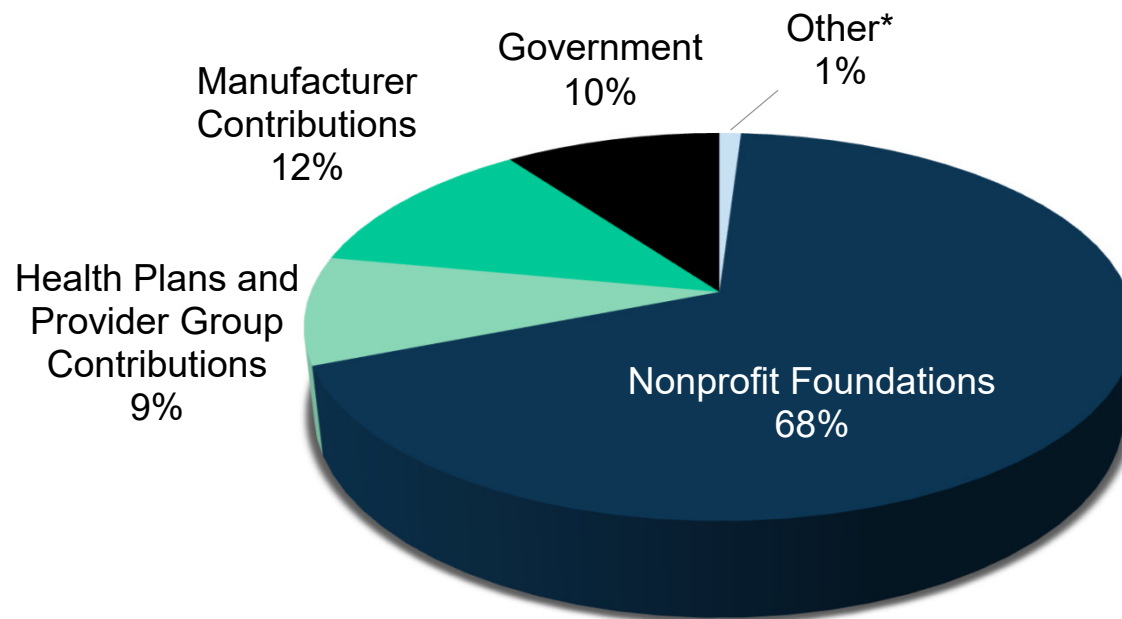
What CAN be Gained from Payer/HTA Involvement in the MULTI-Stakeholder Consensus Development of CORE Outcome SETS for Late Phase Clinical Trials?

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Vice President of Research, ICER



Sources of Funding, 2021

<https://icer.org/who-we-are/independent-funding/>



■ ICER Policy Summit and non-report activities only

*Individual / matching contributions and speech stipends



Perspective

- Health Technology Assessment
- ICER provides an independent source of evidence review to address 3 fundamental questions:
 - How much better is a new treatment at extending or improving patients' lives based on the available evidence?
 - What would a fair price be, based on the clinical evidence as well as patients' perspectives about the outcomes that are most important to them?
 - And how can patients, clinical experts, and insurers translate the evidence into coverage policies that ensures the best patient outcomes?



Reasons for engagement

- The most important benefits and harms of a treatment are those that are important to patients and their families/caregivers
 - We want to align our review with what's important to patients and other stakeholders, but often good quality data doesn't exist to do that
 - Development and adoption of core outcome sets promote generation of more high-quality data that is relevant



Reasons for engagement

- Consistent outcomes allows HTA bodies to compare one treatment to the other
 - Based on the evidence, how much better is a new treatment?
 - Based on evidence, how does it work for different subgroups of patients?



Reasons for engagement

- Provides an opportunity to suggest evidence needs that are important for health technology assessment
- coreSCD
 - Surrogate outcomes vs. Patient important outcomes
 - Difficult to quantify outcomes
 - Quality of life tools



Concluding Reflections

- COS development process provides an opportunity for all stakeholders to be engaged early on in deciding the best evidence to generate
 - Allows different stakeholders to learn from one another
 - Increases the likelihood of researchers to capture a minimum set of outcomes that will be patient-centered and at the same time useful for regulatory bodies, HTA bodies, and clinical decision-makers

Jill Morrow-Gorton, MD, MBA

Medical Director
UPMC Health Plan



COI Statement

Nothing to declare.



Role of State and Federal Payers (Medicare/Medicaid)

- **Quadruple aim—optimize health system performance**
 - Improve health of populations
 - Enhance patient care experience
 - Reduce costs by ensuring clinically appropriate testing and treatment
 - Improve work life for providers
- **Think about cost and quality—value**
 - Provide high-value healthcare (Reuters)
 - Meaningful therapeutic value to the patient considers positive impact on quantity of life, productivity, and fundamentals of the disease state
 - Resource shift to less acute settings to identify who is at risk for progression to avoid
 - Off-sets payer risks
 - High quality care at the lowest cost
 - Stewards of taxpayers' money
- **Role of Value based purchasing/Value based payment (VBP)**

Example of Inconsistent Measurement

Cochrane meta-analysis of use of corticosteroids in Duchenne muscular dystrophy

- 12 studies
 - Steroid vs placebo
 - Steroid daily vs weekend use
 - Different steroid compounds
- What measured (no consistency)
 - Timed tests (timed walk or run, time to stand, stair climb)
 - Ability to lift weights
 - Grading of leg functioning
 - Breathing muscle strength
 - Various adverse events—weight gain, abnormal behavior, appearance changes, etc.
- Timing of studies—6 mos, 12 mos, 24 mos

Approaches to Improving CER

Patient Centered Outcomes Research Institute (PCORI)

- Funds and supports CER that engages and is guided by patients, caregivers, clinicians, researchers and other members of the broader healthcare community
- Leads to individual studies that ask the right questions, include outcomes that matter most to patients, and produce useful and relevant results
- **But does not ensure that important outcomes are consistently measured across studies in a particular disease area**

Center for Medical Technology Policy (CMTP)

- Leads multi-stakeholder development of meaningful, patient-important core outcome sets (COS) in specific disease areas
- Consistent use of COS allows for comparison and synthesis of research results across studies
- Applicable to a body of work related to a topic of research such as sickle cell disease or asthma

How Core Outcome Sets Support Decision Making

Who

- Patient/patient-provider autonomy and shared decision making
- Health plan policy
- State Medicaid program
- Medicare program

Principles

- Outcomes
 - Balance of importance to patients and science of measuring—can it be measured? (e.g. fatigue)
 - Compare choices to assist with informed choice—benefits, risks,
 - What matters to whom
 - Are differences really meaningful

Take Aways

- Patients make it real—what does life look like with disease condition, what is important for quality of life, what is important for better health (including more broadly than specific disease condition)
- Core outcome sets allow meaningful comparison of treatments that informs decisions for treatment of individuals, setting of medical policy and guides for structuring utilization and prior authorization
- Balance which is where value comes in as need to balance quality and cost
 - Value-based purchasing/payment including alternative payment models, value-based contracting, pay for performance
 - Core outcome sets developed through partnerships between clinicians/health technology assessors, patients, payers contribute to more rationale decision making related to the coverage and management of utilization of healthcare interventions

ISPOR Panel



Emily Tsiao, PharmD
Clinical Pharmacist – Utilization Management
Premera Blue Cross

PREMERA

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	Advisory Board	Consultant	Grants/ Research	Salary/ Contractual	Supported Promotional Education	Stock/ Shareholder	Other Financial Support
Emily Tsiao	<i>None</i>	<i>None</i>	<i>None</i>	Premera (employee)	<i>None</i>	<i>None</i>	<i>None</i>

What were you able to bring to the project?

- Regional US payer perspective
- Experience in the following areas
 - Benefit design
 - Value assessment/re-assessment
 - P&T committee management

What were you able to take away? Should more payers/HTA participate in COS development, why or why not?

- Better understanding of the patient experience through discussion with patient/patient advocates in a small group setting
- Rare opportunity to share managed care processes with patients/patient advocates

What is the potential impact of COS for future evidence-based policy-making?

- COSs can help standardize value-based agreements
 - Mutually agreed upon, validated, reliable, meaningful measures help accelerate progress in the realm of value-based arrangements and would support broader uptake of these arrangements
 - Real-world use of a COS in value-based arrangements may help inform future revisions of the COS