Welcome!

Please be seated by 8:50 am ET

The teleconference will go live at 9:00 am ET

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PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Clinical Effectiveness and Decision Science

Advisory Panel Meeting

May 2nd, 2018

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Meetings & Events

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Advisory Panel on Clinical Effectiveness and Decision Science Spring 2018 Meeting

↓

Register Online

Housekeeping

- Today's webinar is open to the public and is being recorded
  - Meeting materials can be found on the PCORI website
  - Comments may be submitted via email to advisorypanels@pcori.org
  - Comments may be submitted via chat; No public comment period is scheduled
- For those in the room, please remember to speak loudly and clearly into a microphone. State your name and affiliation when you speak.
- Where possible, we encourage you to avoid technical language in your discussion

Conflict of Interest Statement

Disclosures of conflicts of interest of members of this Committee are publicly available on PCORI's website and are required to be updated annually. Members of this Committee are also reminded to update conflict of interest disclosures if the information has changed by contacting your staff representative.

If this Committee will deliberate or take action on a manner that presents a conflict of interest for you, please inform the Chair so we can discuss how to address the issue. If you have questions about conflict of interest disclosures or recusals relating to you or others, please contact your staff representative.

Panel Member Introductions

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Agenda Overview

Time	Agenda Item
9:00 – 9:15 am	Welcome, Introductions, Overview of CEDS Advisory Panels
9:15 – 10:00 am	PCORI's APDTO Portfolio and the History of the APDTO Panel
10:00 – 10:45 am	PCORI's CDR Portfolio and the History of the CDR Advisory Panel
10:45 – 11:30 am	Making Research Useful: Identifying and Preventing Methodological Problems
11:30 am – 12:30 pm	Lunch (Acknowledgement of Panelists Last Meeting)
Small/Large Group Discussion Sessions	
12:30 – 12:45 pm	Orientation to Small Group Discussions
12:45 – 2:00 pm	Small Group Sessions: Discuss Priorities for PCORI's Research Programs in the Next 2 Years
2:00 – 2:15 pm	Break
2:10 pm – 3:00 pm	Large Group Discussion: Report Back from the Small Group Discussions
3:00 – 3:15 pm	Wrap-Up, Next Steps, Debrief
3:15 pm	Adjourn

Welcome and Overview of CEDS Advisory Panel

David Hickam, MD MPH

Program Director, Clinical Effectiveness and Decision Science

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History of PCORI Advisory Panels

Context and Objectives

- In 2012, the PCORI BoG announced 5 National Research Priorities.
  - Assessment of Prevention, Diagnosis and Treatment Options
  - Communication and Dissemination Research
  - Improving Methods and Infrastructure
  - Improving Healthcare Systems
  - Addressing Disparities
- Advisory panels provide stakeholder input to guide the direction of the research priority areas.
  - 2013: APDTO Advisory Panel
  - 2015: CDR Advisory Panel

Joint CDR / APDTO Panel

Context and Objectives

- In 2016 the PCORI Science Program underwent reorganization to align the national research priorities with programmatic functions and structure:
  - Clinical Effectiveness and Decision Science (APDTO, CDR, Methods)
  - Healthcare Delivery and Disparities Research (IHS, Disparities)
- In December 2017, the BoG merged the APDTO and CDR Panels.
  - CEDS Advisory Panel
- Our goal is to develop a unified vision for the research priority areas that is informed by the needs and perspectives of stakeholders.

Introductions

Lauren McCormack, PhD, MSPH

Vice President, Public Health Research Division, RTI International

Representation on CDR Advisory Panel: Panel Chair

Formerly: *CDR Advisory Panel Co-Chair*

Currently: *CEDS Advisory Panel Co-Chair*

Danny Van Leeuwen, Opa, RN, MPH, CPHQ

Owner, Health Hats

Representation: Patients, Caregivers, and Patient Advocates

Formerly: *CDR Advisory Panel Co-Chair*

Currently: *CEDS Advisory Panel Co-Chair*

Michael Herndon, DO

Chief Medical Officer, Oklahoma Health Care Authority

Representation on APDTO Advisory Panel: Payer

Formerly: *APDTO Advisory Panel Member*

Currently: *CEDS Advisory Panel Member*

Clinical Effectiveness Research

The evolution of PCORI's CER portfolio

Kim Bailey, MS

Program Officer, Clinical Effectiveness and Decision Science

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Goals for Presentation

- Review PCORI's portfolio of clinical effectiveness research projects across the following portfolios
  - ✓ Assessment of Prevention, Diagnosis, and Treatment Options
  - ✓ Pragmatic Clinical Studies
  - ✓ Targeted Funding Announcements
- Discuss where the programs have been and how they have evolved over time

First Out of the Gate:

The Assessment of Prevention, Diagnosis, and Treatment Options (APDTO) Funding Announcement

APDTO

Purpose of Funding Announcement

- Goal of the program is to fund investigator-initiated research that
  - Compares the effectiveness of two or more strategies for prevention, treatment, screening, diagnosis, or management
  - Compares specific clinical services or strategies that are clearly defined and can be replicated in other clinical settings with minimal adaptations or changes
- Funding announcement does not support
  - Projects with the primary goal of developing and testing decision aids
  - Projects testing the use of lay personnel who perform ancillary services in healthcare settings

APDTO

Program Overview

- Cycles: Cycle 1 2018 is the 14th release
- Funds Available: Historically, up to $32M per cycle and up to $2M in direct costs per project
- Duration: Typically 36 months
- Recent Addition: Small and Large study mechanisms ($2M direct costs, 3 year max. duration; $5M direct costs, 4 year max. duration)
- Projects Awarded: 119 through Cycle 1 2017
- Funds Awarded: Roughly $240M through Cycle 1 2017
- Award amounts: ~$700k– 6.7M in total costs
  - Median total costs of ~$2.0M
- DFRRs submitted: 63 as of 4/23/18

APDTO

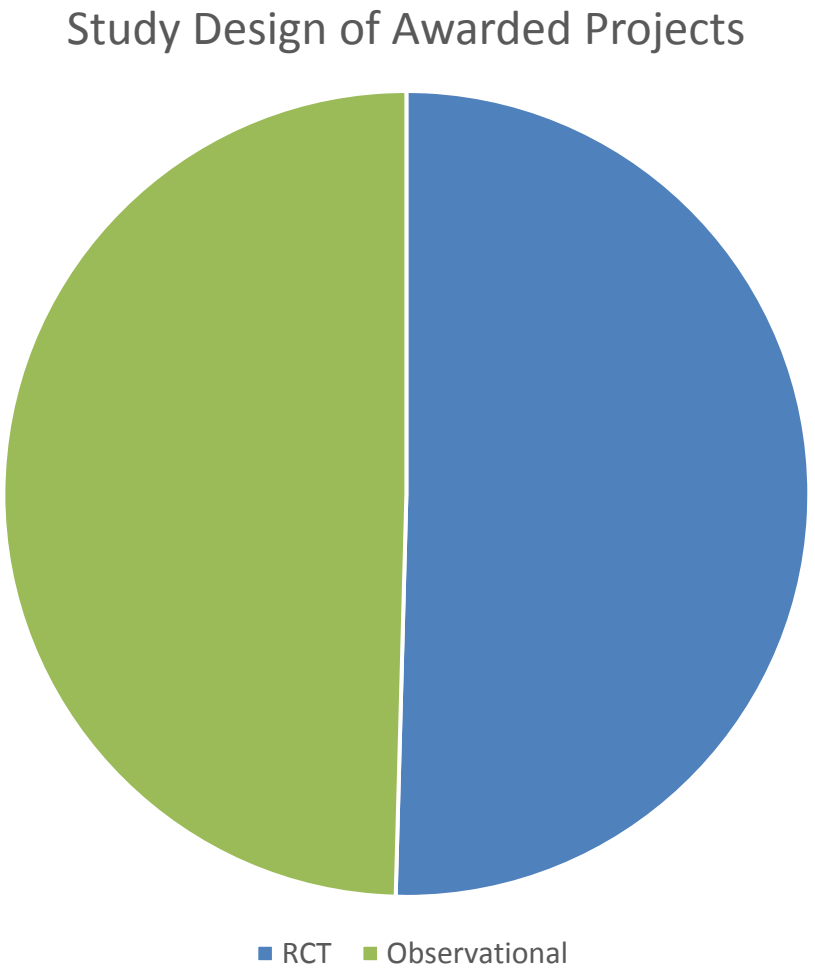
Current Portfolio: Primary Clinical Conditions Explored

- Very broad range of clinical topics
- Top 3 conditions explored:
  - Cancer – 26 studies
  - Mental and behavioral health – 13 studies
  - Cardiovascular diseases – 12 studies
- Focus on rare diseases – 9 studies

APDTO

Current Portfolio: Study Design

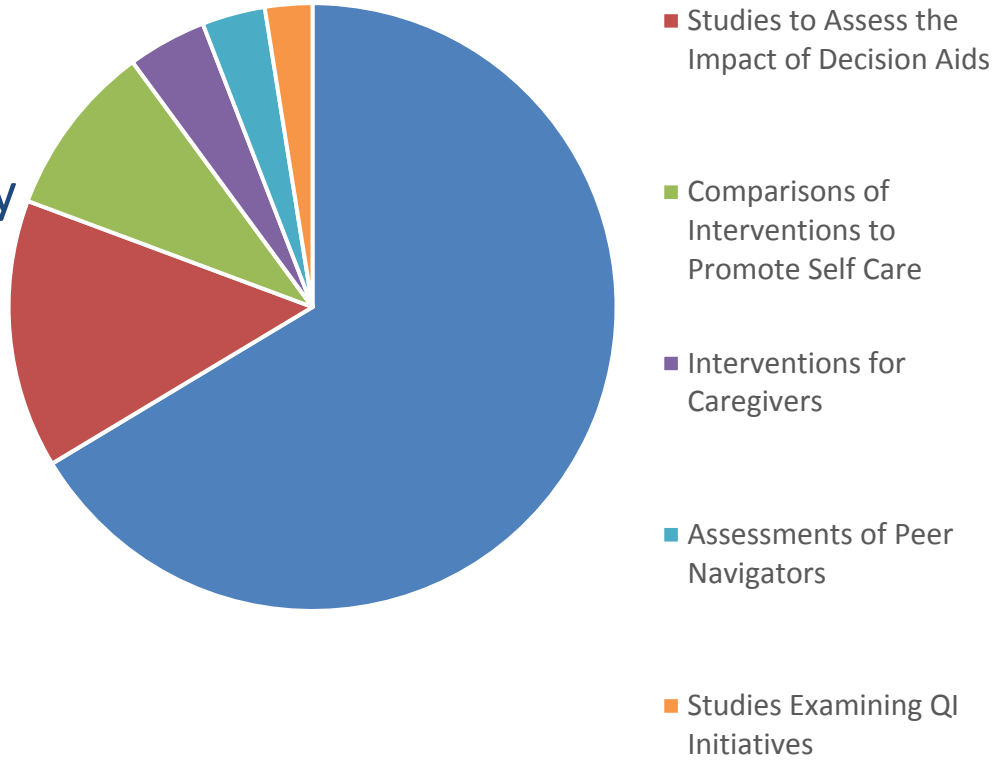
- Nearly even split between RCTs and observational designs
- For the RCTs, sample sizes range from 86 to 1,833 patients (Mean: 443; Median: 300)



APDTO

Current Portfolio: Intervention Type

- More than 2/3 of the APDTO portfolio includes comparisons of clinical strategies
- The proportion of APDTO studies focused on comparisons of primary clinical strategies has increased in recent cycles
- Projects focused on QI efforts, assessments of decision aids, and assessments of the impact of peer navigators were awarded in early cycles



Refining the Vision:

The Pragmatic Clinical Studies Program

Pragmatic Clinical Studies

Background and Purpose

- Program launched in early 2014 to expand support of high-priority patient-centered comparative clinical effectiveness research
- Funds large pragmatic clinical trials, large simple trials, or large-scale observational studies that compare two or more alternatives for addressing:
  - Prevention, diagnosis, treatment, or management of a disease or symptom, or
  - Improving healthcare system-level approaches to managing care, or
  - Communicating or disseminating research results to patients, caregivers, or clinicians, or
  - Approaches to eliminate health disparities
- Funded studies must address critical clinical choices faced by patients, caregivers, clinicians, or delivery systems
- Funded studies must involve broad patient populations and be able to provide precise estimates of hypothesized effectiveness differences

Pragmatic Clinical Studies

Current Portfolio: Overview

- The PCS PFA has been released 11 times (from Spring 2014 through Cycle 1 2018)
- As of mid-2018, there are 31 awarded projects in the portfolio, amounting to nearly $365M to date
- Of the 31 studies, 18 are clinical comparisons managed by the CEDS program and 13 are health systems comparisons managed by the HDDR program
- Budget: $7.5M – 18.5M in total costs (generally limited to $10M in direct costs)
- Duration: 5 years to 7.5 years (includes peer review)
  - Earliest results will be available in 2020

Pragmatic Clinical Studies

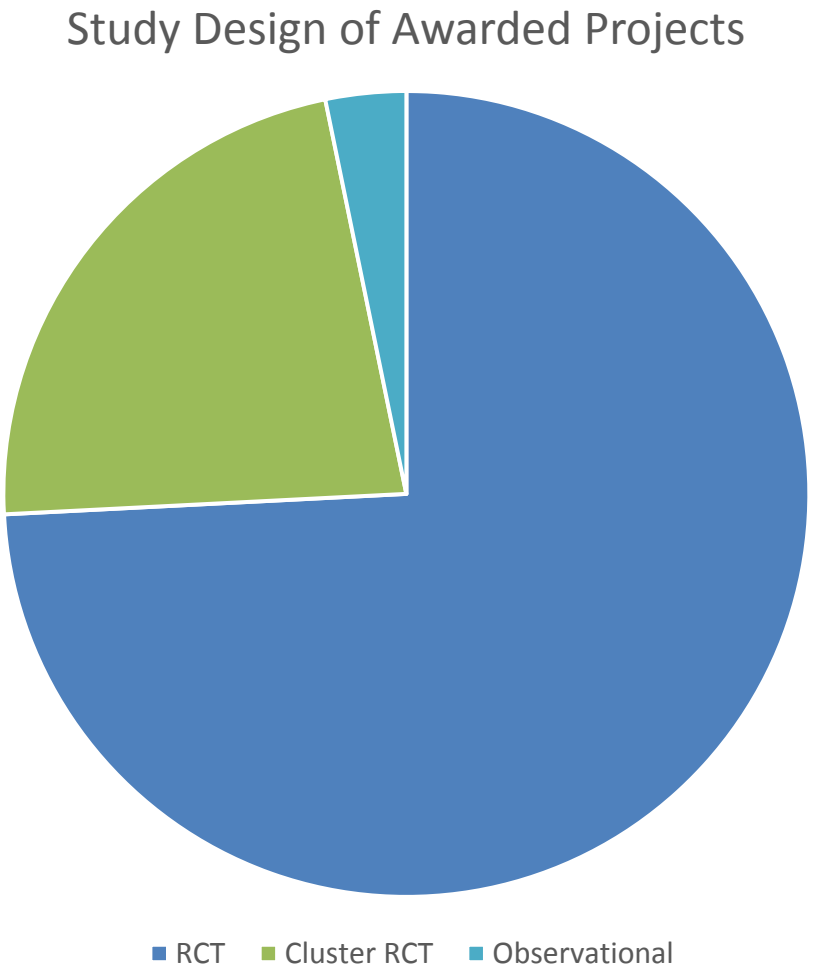
Current Portfolio: Primary Clinical Conditions Explored

- Cancer – 8 studies
- Mental and behavioral health – 5 studies
- Muscular and skeletal disorders – 4 studies
- Cardiovascular diseases – 3 studies
- Gastrointestinal disorders – 2 studies
- Respiratory diseases – 2 studies
- Infectious diseases – 2 studies
- Other conditions – 5 studies

Pragmatic Clinical Studies

Current Portfolio: Characteristics of Awarded Projects

- Designs consist of mostly RCTs, with cluster RCTs and one observational study
- For the RCTs, sample sizes range from 500 to 65,000 patients
- The one observational study aims to review the scans of 1 million women (approximately 2.8 million scans)



Homing in:
Targeted Funding Announcements

Targeted Funding Announcements

Background and Purpose

- Program launched in Spring 2015 in an effort to target funding toward topic areas of particular interest to PCORI's stakeholders
- Targeted funding announcements, including the specific research questions of interest, are developed in partnership with key stakeholders
- Successful proposals must be responsive to the questions defined in the targeted funding announcement

Targeted Funding Announcements

Current CER Targeted Portfolio: Overview

- As of May 2018, the CEDS team has managed the release of 11 targeted funding announcements
- Awards have been announced in 8 of the 11 funding announcements
- 21 studies have been awarded through these seven funding announcements
- Budget: $2.0M – 15.5M in total costs
- Duration: Most are 5 year studies
  - Earliest results will be available in 2021

Targeted Funding Announcements

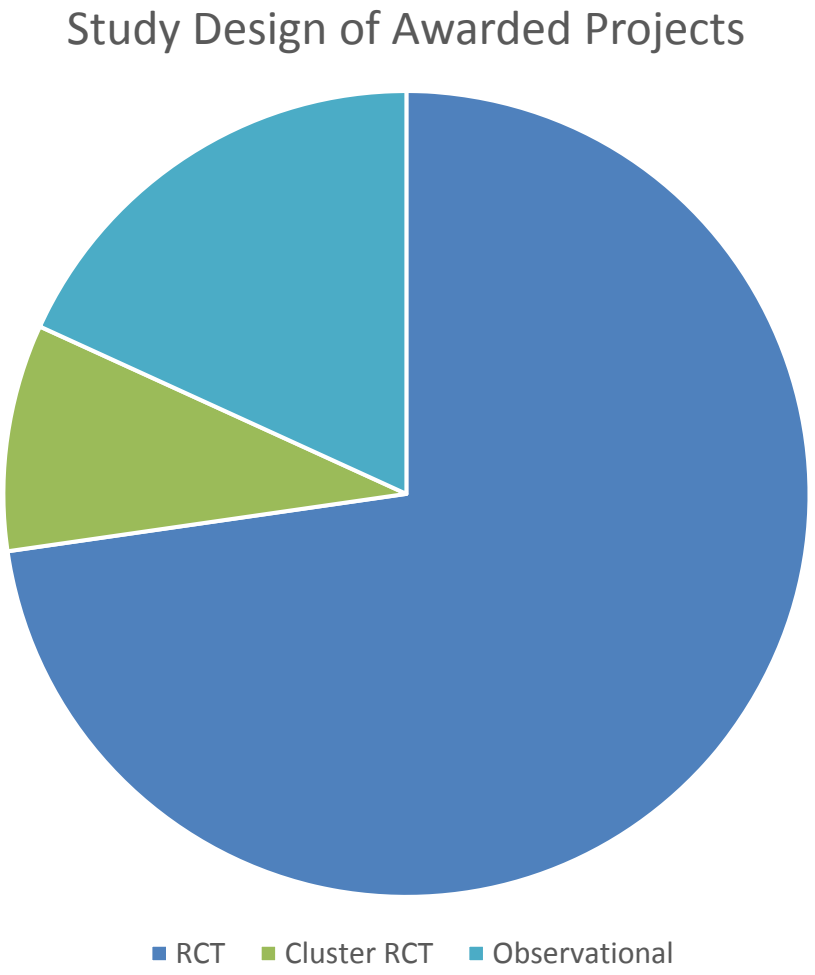
CER Targeted Announcements, to date

- Clinical management of hepatitis C (2015, 2 studies)
- Treatment of multiple sclerosis (2015-16, 9 studies)
- Management strategies for treatment-resistant depression (2015, 3 studies)
- New oral anticoagulants (NOACs) in the extended treatment of VTE (2015, 3 studies)
- Clinical strategies for managing and reducing long-term opioid use for chronic pain (2015-16, 4 studies)
- Comparison of surgical and nonsurgical options for management of nonspecific chronic low back pain (2017, 1 study)
- Symptom management for patients with advanced illness (2017, 1 study)
- Pharmacological treatment for anxiety disorders in children, adolescents, and/or young adults (Cycle 1 2018)

Targeted Funding Announcements

Current CER Portfolio: Characteristics of Awarded Projects

- More than 3/4 RCTs with randomization at patient-level
- Four observational studies, all large with the ability to examine subgroups of interest
- For the RCTs, sample sizes 136 to 3,165 (median: 810)



Conclusions

- The overall portfolio of funded projects includes a broad range of patient populations and clinical issues
- PCORI has become increasingly targeted in its funding announcements
- The studies use a broad range of study designs and data sources
  - A slight majority of funded studies are randomized controlled trials
- PCORI has moved towards an emphasis on specific clinical issues
  - Clearly influenced by prioritization activities of APDTO Advisory Panel
  - PCS priority topic list
  - Targeted funding announcements

In Retrospect

The history of the APDTO Advisory Panel

Mike Herndon, DO

Former APDTO Panel Member

Current CEDS Panel Member

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History of APDTO Priority

- Direct comparison of specific clinical services
  - Benefits and harms of those services
- Emphasis on patient-centered clinical outcomes
  - Symptoms
  - Functioning
- Original PFA for the APDTO Priority issued in 2012
- Funded 155 projects through 2017
  - Most examine treatments for specific diseases

History of APDTO Advisory Panel

- APDTO Advisory Panel first met in April 2013
- 14 meetings through 2017
- Purpose: to "advise and provide recommendations to PCORI's Board of Governors, Methodology Committee, and staff to help plan, develop, implement, improve, and refine efforts toward meaningful patient-centered research"
  - Prioritize critical research questions for possible future funding
  - Provide ongoing feedback and advice on evaluating and disseminating the research conducted under this priority
- As of today's meeting, the APDTO panel has reviewed 84 clinical effectiveness research topics

How the APDTO Advisory Panel Performed its Work

- In early meetings, the panel reviewed a large number of topics based on brief synopses.
  - Provided broad priority areas when PCORI had not yet defined priorities
- Panel members served as “topic reviewers” for individual topics.
- Transition to more in-depth topic reviews
  - Fewer topics discussed at each meeting
  - Detailed topic summaries and literature reviews
  - Focus on evidence gaps within the topic areas
- Feedback to panel: updates about prior topics at the next meeting

High Priority Topics in Pragmatic Clinical Studies Program

2018 Priority Topic List

- **Treatment of anxiety in children, adolescents and young adults**
- **Comparing the benefits and harms of treatment strategies for various types of insomnia**
- **Treatment of community-acquired pneumonia**
- **Second-line treatments for non-muscle invasive bladder cancer**
- **Surgical options for hip fracture**
- **Treatment strategies for symptomatic osteoarthritis**

APDTO AP-Reviewed Topics placed on PCS Priority List

Prior Topics Included in PCS Priority List	Project Results
Treatment options for hepatitis C infection	***Issued as tPFA***
Treatment programs for recurring/remitting multiple sclerosis	***Issued as tPFA***
Medication regimens, intensive counseling, and combined modalities for treatment of opioid substance abuse	***Issued as tPFA***
Treatment of anxiety in children, adolescents, and young adults	***Issued as tPFA***
Antipsychotic use for bipolar disorder in children, adolescents and young adults	***Project Funded***
Management strategies for ductal carcinoma in situ (DCIS)	***Project Funded***
Treatment strategies for adults with frequent migraine headaches	***Project Funded***
Diagnostic modalities for identifying lung cancer in people with lung nodules.	***Project Funded***

APDTO AP-Reviewed Topics placed on PCS Priority List (cont.)

Prior Topics Included in PCS Priority List	Project Results
Proton beam therapy for breast, lung, and prostate cancer	***Projects Funded***
Use of biologics to treat inflammatory diseases, including Crohn's disease, ulcerative colitis, and rheumatoid arthritis	***Project Funded***
Surgical options for hip fracture in the elderly	***Related Project Funded***
Treatment options for autism	***Topic Retired***
Renal replacement therapies for patients of different ages, races, and ethnicities	***Topic Retired***
Medical and surgical treatment options for patients with asymptomatic carotid artery stenosis	***Topic Retired***
Benefits and harms of pelvic floor mesh Implants	***Topic Retired***

Update on Eating Disorders Topic

November 2017: Topic Brief and Discussion by ADPTO Advisory Panel

January 2018: Key informant interviews with professional societies and individual experts

March-April 2018: Discussions with Scientific Oversight Committee

Draft Language for Eating Disorders Topic

- Compare evidence-based approaches for treatment of anorexia nervosa or bulimia nervosa in adolescents and/or young adults (through age 25)
  - Studies that expand the evidence to support feasible, acceptable, and broadly available treatments for patients in outpatient care.
  - Studies that compare different approaches to treatment initiation, sequencing, caregiver support, and/or relapse prevention following an initial effective course of treatment.
  - PCORI encourages investigators to ascertain a full range of clinical, caregiver- and patient-reported outcomes with at least 1 year of follow-up.
- Timetable for new priority topics or targeted funding announcements is uncertain.

Track Record of APDTO Advisory Panel

- Active panel that held 3-4 meetings per year
- Developed a successful approach to review and prioritize nominated clinical topics
- Success in moving high priority projects forward
  - Targeted funding announcements
  - Funded projects that aligned with priority clinical topics

Questions/Comments?

Communication and Dissemination Research

Bridget Gaglio, PhD, MPH

Senior Program Officer

Clinical Effectiveness and Decision Science

Agenda for Presentation

Future Directions

Current CDR Portfolio

Background and history of CDR PFA

Why is CDR important?

- Knowledge needs to be strengthened about how to communicate optimally and facilitate the effective use of patient-centered outcomes research (PCOR) and comparative clinical effectiveness research (CER) findings by patients, caregivers, and healthcare professionals.
- Well-documented barriers exist to the rapid transfer of evidence that could be useful in decision-making.
- Informed healthcare decisions require innovative and effective strategies to make existing PCOR/CER evidence available to patients and providers in real-world settings.
- Moreover, the information needs to be understandable to improve decision making.

Clear communication approaches and active dissemination of PCOR/CER research findings to all audiences are critical to increasing the awareness, consideration, adoption, and use of these data by patients, caregivers, and healthcare providers.

Terminology and Definitions

Concept or Construct	Definition As It Relates to Health and Health Care
Health Communication Research	The study and use of communication strategies to inform and influence individual, provider, and community decisions that affect health.
Dissemination	An active approach of spreading evidence-based interventions to the target audience via determined channels using planned strategies. The intent is to spread ("scale up") and sustain. *Engagement*
Dissemination Research	The systematic study of processes and factors that lead to widespread use of an evidence-based intervention by a target population. Its focus is to identify the best methods that enhance uptake and utilization of the intervention. *CDR*
Implementation Research	The scientific study of methods to promote the integration of research findings and evidence-based interventions into healthcare practice and policy.

Difference between ADPTO and CDR

- *Assessment of Prevention, Diagnosis, and Treatment Options*
  - Focused on adding to the evidence base to make the best choices regarding, prevention, diagnosis, and treatment. Comparison of clinical options not only in terms of effectiveness, but including outcomes important to patients and for subpopulations.

- *Communication and Dissemination Research*
  - How best to communicate and disseminate the evidence so patients and providers are aware of the evidence and can use the information in the context of their personal characteristics, conditions, and preferences when making treatment choices.

CDR
Purpose of Funding Announcement

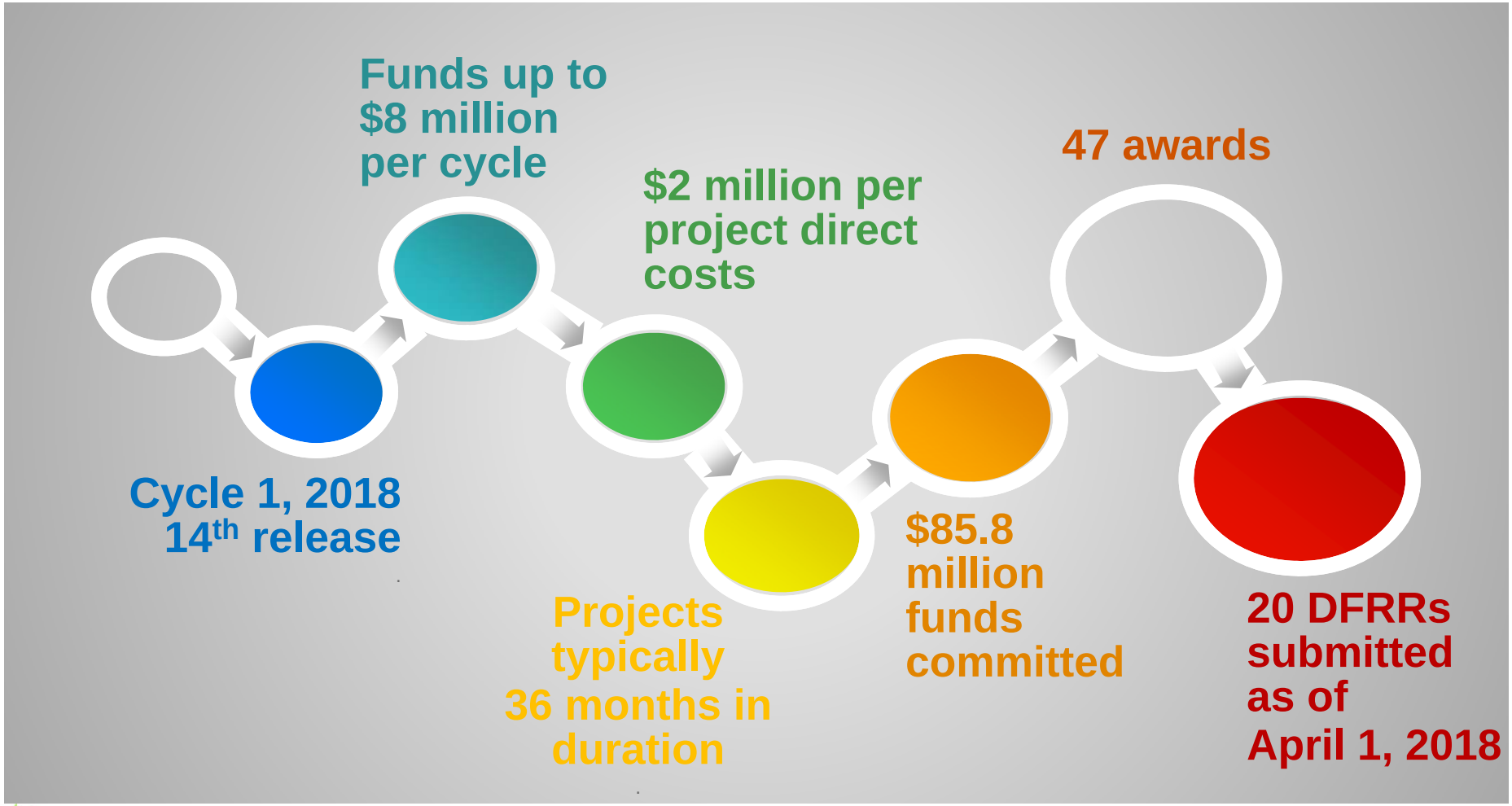
The CDR PFA invites investigator-initiated research that study the comparative effectiveness of communication and dissemination strategies.

- ***The PFA focuses on three key areas:***
  - *communication strategies to promote the use of health CER evidence,*
  - *dissemination strategies to promote the use of health CER evidence,*
  - *and explaining uncertain health CER evidence.*

- Funding announcement does not support

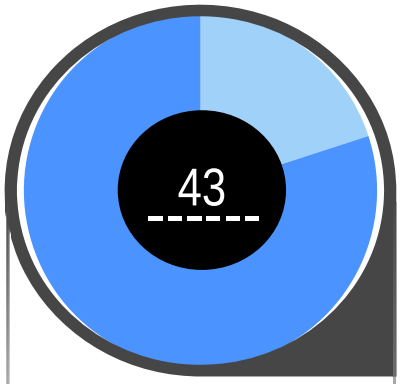
  While we are interested in understanding the role of shared decision making and established, effective decision aids in communicating and implementing PCOR/CER. Applications focused on developing, testing (establishing efficacy), and validating individual decision aids and tools are considered nonresponsive to the CDR PFA.

CDR
Portfolio Overview

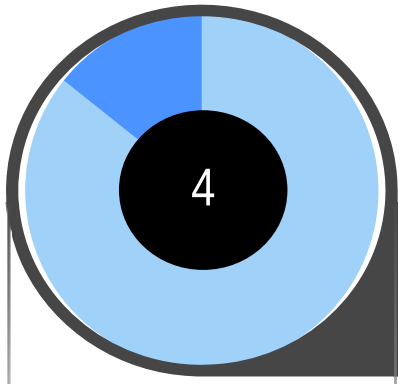


CDR

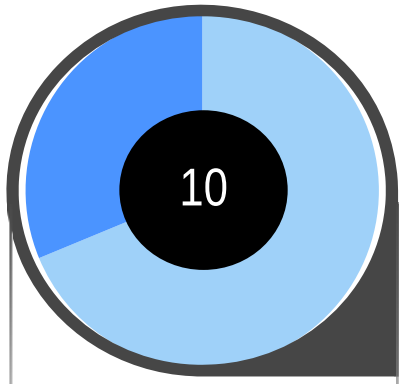
Current Portfolio: Areas of Emphasis



- Communication Strategies



- Dissemination Strategies



- Explaining Uncertainty

* Categories not mutually exclusive

Focus of Communication Strategies Studies

Category	N
Decision aid compared to usual care Interactive (e.g., mobile applications, web-based) Paper (e.g., graphic novels, questions prompt lists)	 15 6
Decision aid compared to decision aid(s)	4
Provider training	4
Sharing of information compared to usual care E.g., self-management education, peer/navigator, reports	6
Sharing of information intervention compared to sharing of information intervention	3
Other	5

Examples of Communication Strategies Studies

- **Decision aid compared to decision aid(s) -**
  - Shared decision making about contraceptive methods for female patients receiving care at 16 ethnically diverse U.S. clinics that deliver contraceptive care.
    - 1) video + prompt card
    - 2) option grid decision aids + training for health care providers
    - 3) video + prompt card and decision aids + training
    - 4) usual care
  - Decision quality at 1 week and 12 months in adult patients considering total joint replacement for hip or knee osteoarthritis.
    - long patient decision aid (PDA) plus enhanced report for surgeon that provides patients' goals and treatment preferences
    - short PDA plus enhanced report
    - long PDA plus standard surgeon report
    - short PDA plus standard surgeon report

Examples of Dissemination Research Studies

- **Management of Chronic Illness in Pediatric and Adult Patients-**
  - Medicaid patients' perception of shared decision making during asthma care
    - Asthma Shared Decision Making Toolkit using the *F*acilitator *L*ed, participant *OW*ned (*FLOW*) dissemination approach
    - Traditional (lunch and learn) dissemination
    - No dissemination
- **Management of Chronic Illness in Pediatric Patients-**
  - Parent report of decisional uncertainty and perception of shared decision-making about hydroxyurea use for young children (0-5 years of age) with sickle cell disease.
    - Hydroxyurea Shared Decision Making (H-SDM) toolkit
    - Clinician pocket guide

Examples of Explaining Uncertainty Studies

- **Self-management of Chronic Illness –**
  - Informed decision making at 6-months in adults with rheumatoid arthritis.
    - DrugFactsBoxes® with and without gist reasoning training to enhance patient understanding of medication risks/benefits
    - FDA-mandated Medication Guides with and without gist training
- **Management of Acute Pain –**
  - Use of opioids at 14 days for acute renal colic or back pain.
    - Narrative Enhanced Risk Tool (NERT)
    - Probabilistic Risk Communication (PRT)
    - Generalized Risk Communication (GRC) only
- **Screening/Prevention-**
  - Screening intention, screening behavior, and perceptions for patients eligible for colorectal cancer screening.
    - Decision aid with quantitative information
    - Decision aid without such data

Involvement in Special Areas of Emphasis

CDR involvement to date

- *Strategies to Prevent Unsafe Opioid Prescribing in Primary Care among Patients with Acute and Chronic Noncancer Pain*
  - Comparative effectiveness of different patient- and provider-facing interventions that facilitate improved knowledge, communication, and shared decision making about the relative harms and benefits of opioids and alternative treatments on prevention of unsafe prescribing and improved patient outcomes?

- *Community-Based Palliative Care Delivery for Adults with Advanced Illnesses and their Caregivers*
  - Comparative effectiveness of different patient and caregiver-directed, clinician-directed, and combination approaches to facilitating advance care planning conversations between adult patients living with advanced illnesses, their caregivers, and clinicians on patient-centered and other outcomes over time?

Future Directions

Questions for discussion

- How can we better articulate what we are seeking to the research community? Especially as it relates to dissemination focused applications.

- Are these three areas of emphasis still relevant?

In Retrospect

The history of the CDR Advisory Panel

Lauren McCormack, MPH, PhD

Former Chair, CDR Advisory Panel

Current Chair, CEDS Advisory Panel

Danny van Leeuwen, MPH, RN, CPHQ

Former Co-Chair, CDR Advisory Panel

Current Co-Chair, CEDS Advisory Panel

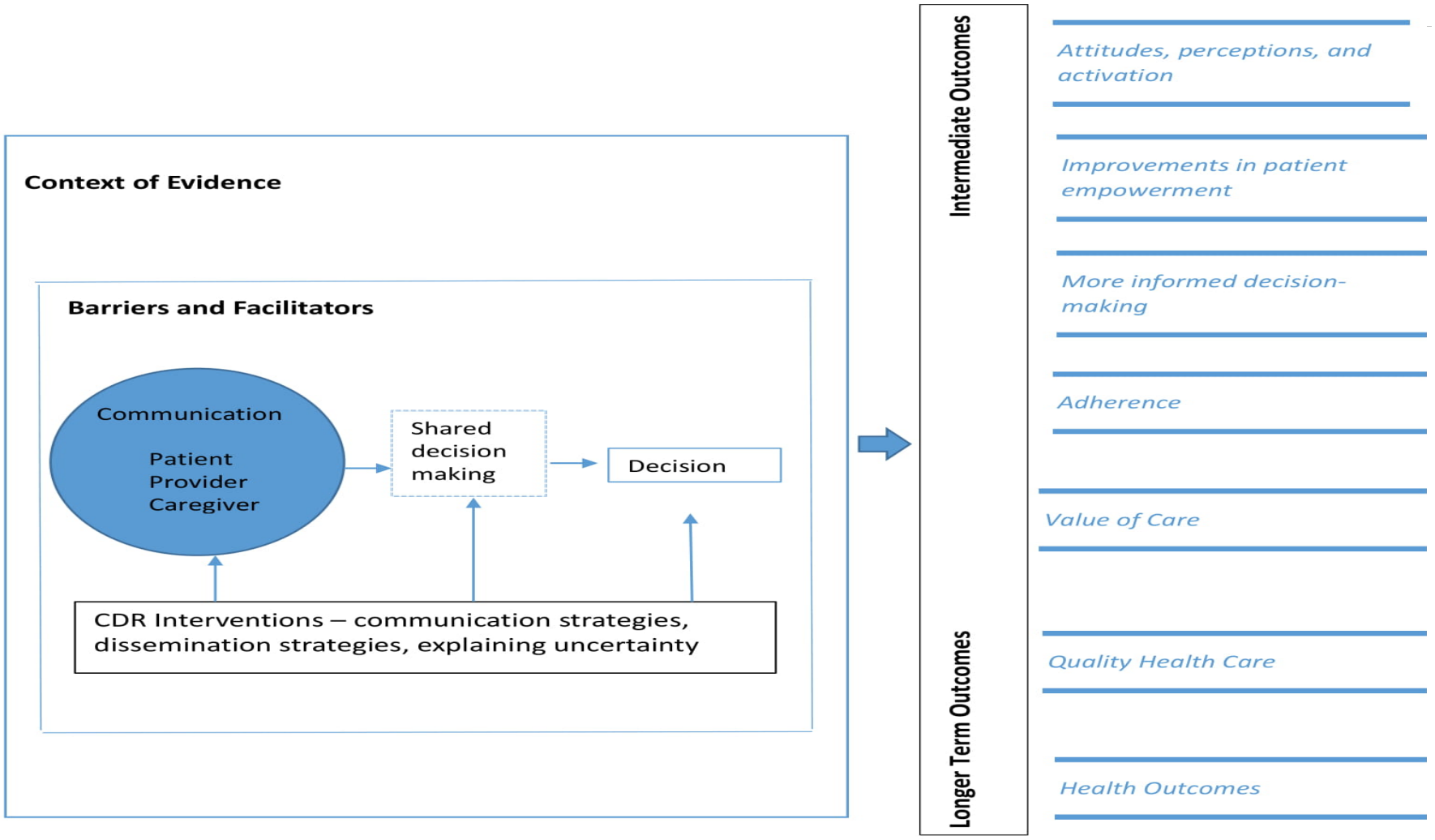
CDR Activities & Accomplishments

- Clarified terminology and understanding of CDR goals & objectives
- Identified outcomes to be measured
- Developed a framework to inform Communication, Dissemination and Uncertainty (CDR) research
- Presented the challenges in communicating about uncertainty
- Explored alternative channels for dissemination

Outcomes of CDR:
What does success look like?

- Obtaining patient input and involvement in decision making
- Increased understanding of and appreciation for patients' culture, communication and care needs
- Goal setting and alignment across the health care team
- Adherence to guidelines
- Evidence-based decisions even when the evidence is lacking
- Caregiver involvement
- Increased satisfaction with the decision-making process and the decision
- Decreased provider burnout
- Infusion of innovation

Conceptual Framework of CDR Advisory Panel



Cross-cutting:

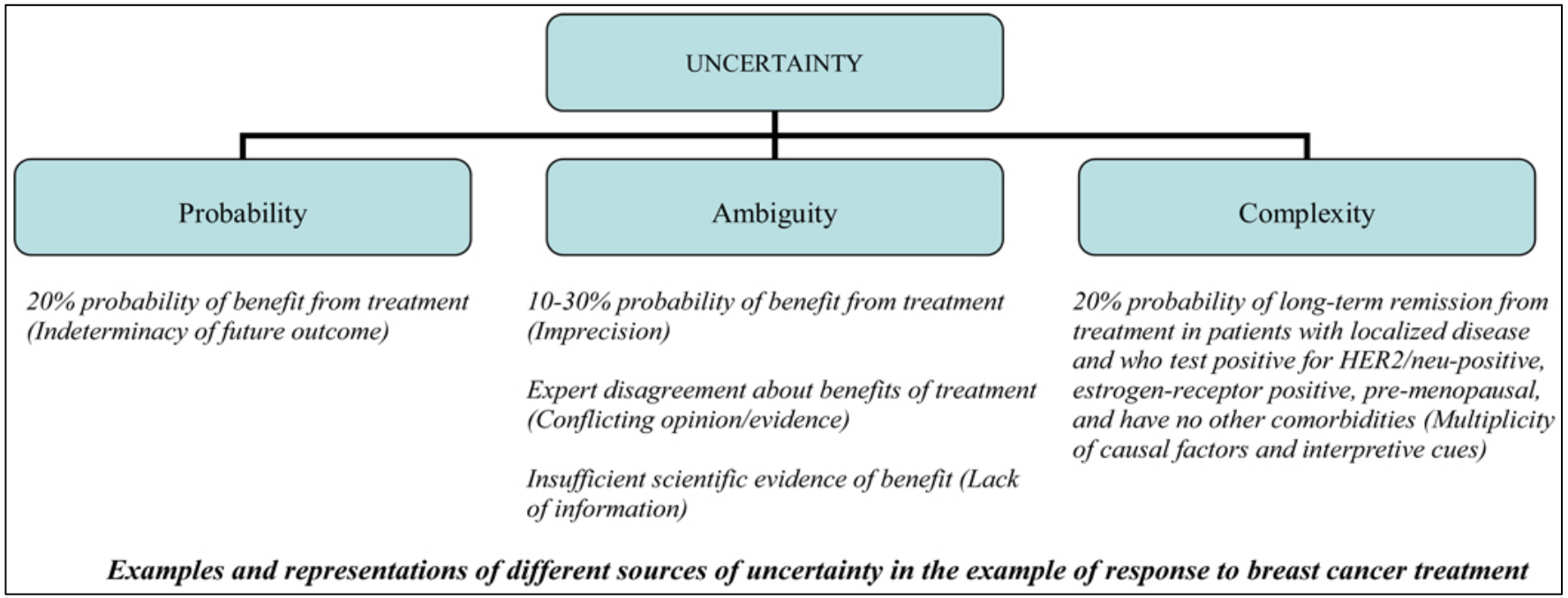
- Time
- Multi-level contextual functions
- Healthcare system

Contents of the Framework Article

- Introduction – context of the PCORI CDR portfolio
- Methods – original literature review supplemented by updated review, advisory panel collaboration process
- Results
  - Framework visual
  - Communication & dissemination strategies
  - Outcomes
- CDR funding mechanism
- Application of the framework in the future

Different Types of Uncertainty

- https://www.ncbi.nlm.nih.gov/core/lw/2.0/html/tileshop_pmc/tileshop_pmc_inline.html?title=Click%20on%20image%20to%20zoom&p=PMC3&id=3146626_nihms284817f1.jpg



Examples and representations of different sources of uncertainty in the example of response to breast cancer treatment

Reference: Paul K.J. Han, MD, MA, MPH,[1] William M.P. Klein, PhD,[2] and Neeraj K. Arora, PhD[2]. (2011) Varieties of uncertainty in health care: a conceptual taxonomy. Med Decis Making. 2011 Nov-Dec; 31(6): 828–838. doi: 10.1177/0272989X11393976

Dealing with Real World Uncertainty:

Bridging population level evidence and personal circumstances and preferences

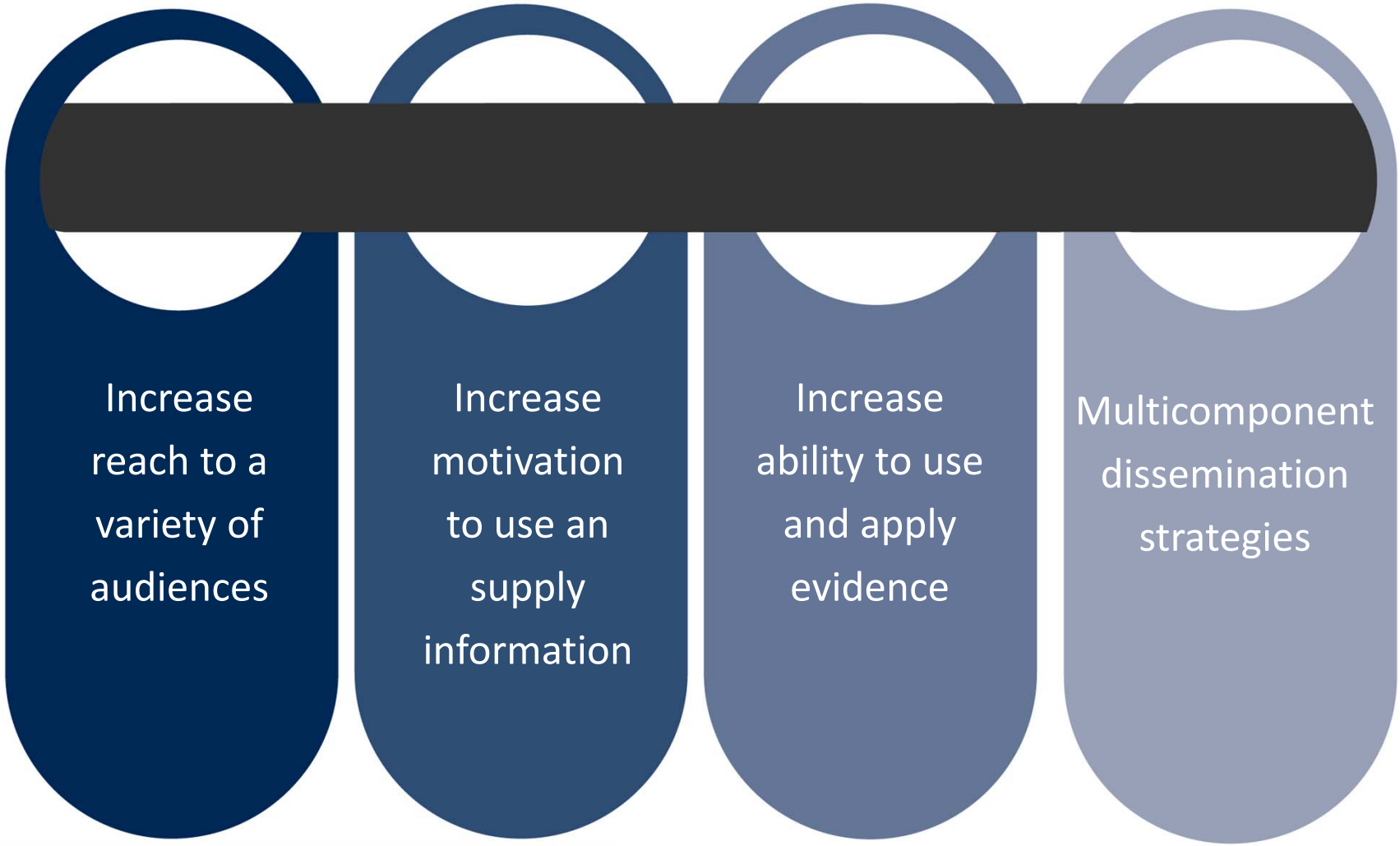
- What challenges have you experienced in your life or your work when speaking, hearing, writing, or thinking about the uncertainty of evidence?
- How do those challenges affect decisions you, your patients, or members of your health team make?
- How do those decisions affect the relationships between you and your patients or members of your health team?
- What research might mitigate any of those challenges?

Uncertainty Concepts Addressed in the Systematic Review

Overall strength of evidence	Degree of confidence that the estimates of effects are correct and represent the true effect. When overall strength of evidence is insufficient or low, uncertainty is high.
Risk of bias	Degree to which individual studies are protected from systematic errors or bias. When risk of bias is high, the quality of evidence is poor, leading to uncertainty.
Consistency	Degree to which studies present findings similar in direction of effect, magnitude of effect, or both. Evidence lacking consistency includes studies with greatly differing or conflicting effect estimates.
Precision	Degree of random error surrounding an effect estimate with respect to a given outcome. Studies express dispersion around a point estimate of risk, such as a confidence interval, which indicates the reproducibility of the estimate.
Directness	Degree to which the evidence either directly links the interventions to the outcome of interest or directly makes the comparison of interest. When evidence indirectly links interventions to the outcomes most of interest, evidence is uncertain.
Net benefit	Balance or tradeoffs in benefits and harms for prevention or treatment services. When the balance of benefit and harm is too close to call or when evidence is lacking, the appropriate course of action with regard to prevention or treatment is uncertain.
Applicability	Whether a study intervention is expected to have the same effect in populations and settings where it was not studied but might be applied.
Overall strength of recommendation	The overall judgment of policymakers that evidence should be applied in particular populations and settings

Internet Citation: Communication and Dissemination Strategies To Facilitate the Use of Health-Related Evidence. Content last reviewed November 2013. Agency for Healthcare Research and Quality, Rockville, MD. http://www.ahrq.gov/research/findings/evidence-based-reports/commstrattp.html

Goals for Dissemination



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Alternative channels for dissemination

- **Social Networks** Keren Ladin, PhD, Tufts University and Director of the Lab for Research on Ethics, Aging, and Community Health (REACH Lab).
  - understand the role of social networks in complex medical decision-making,
  - harness social networks and social support to improve health care utilization among vulnerable populations

- **Prescription2Learn** Sarah Krug, MD, Executive Director of Cancer 101
  - guides patients and caregivers as they explore and vet evidence in their health journey. This resource addresses the increasing access of health information by individuals through social media addressing issues of accuracy and availability.

- **Social Media** Jarred Younger, PhD, Asst Professor at the University of Alabama and Director of the Neuroinflammation, Pain and Fatigue Lab.
  - Using social media to listen to and inform persons with or treating Fibromyalgia. Explained the current state, the limitations, and the potential of research.

Questions/Comments?

Making Research Useful:
Identifying and Preventing Methodological Problems

Emily Evans, PhD MPH

Program Officer, Clinical Effectiveness and Decision Science

David Hickam, MD MPH

Program Director, Clinical Effectiveness and Decision Science

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Goals of this Presentation

- Summarize major trends for assuring the scientific integrity of patient centered outcomes research
- Review the PCORI Methodology Standards and their place in PCORI's process for evaluating research
  - Funding applications
  - Monitoring projects that have received funding

Increasing Value in Research: Research Priorities & Study Questions

- **Research must be justified**
  - Limited resources
  - Imposition of risks

- **To be justified, a particular study must have the potential to generate the evidence needed to make an informed health decision**
  - Compelling decisional dilemma (PCOR/CER)
  - Design, conduct, & analysis
  - Feasibility
  - Ethical permissibility

- **Clinical burden, evidence gaps, and public support are not sufficient to justify a particular study**
  - The study must be appropriately designed to achieve its objectives.

Increasing Value in Research: Study Design & Analysis

- **Study Protocols**
  - Insufficiently detailed, inaccessible, or non-existent
- **Design Considerations**
  - Failure to account for and appropriately address potential sources of bias other than confounding
  - Trying to “trade-off” internal vs. external validity
- **Analytic Approach**
  - Insufficient statistical expertise on the research team and/or insufficient involvement of the statistician
- **Consideration of Other Evidence**
  - Interpreted in isolation, data from an individual study cannot tell us the probability that a hypothesis is true and/or how we should act based on these findings

PCORI's Methodology Standards

- Required by PCORI's authorizing law
- Represent minimal standards for design, conduct, analysis, and reporting of comparative effectiveness research (CER) and patient-centered outcomes research (PCOR)
- Reflect generally accepted best practices
- Used to assess the scientific rigor of applications, monitor the conduct of research awards, and evaluate final research reports

2018 PCORI Methodology Standards (Updated 4/30/2018)

The 54 standards can be grouped into 2 broad categories and 13 topic areas.

Cross-Cutting Standards

- Formulating Research Questions
- Patient Centeredness
- Data Integrity & Rigorous Analyses
- Preventing/Handling Missing Data
- Heterogeneity of Treatment Effects

Design-Specific Standards

- Data Registries
- Data Networks
- Causal Inference Methods*
- Adaptive & Bayesian Trial Designs
- Studies of Medical Tests
- Systematic Reviews
- Research Designs Using Clusters
- Studies of Complex Interventions

The first standard for Causal Inference Methods (CI-1) is considered cross-cutting and applicable to all PCOR/CER studies.

Methodology Standards: Research Priorities & Study Questions*

- **RQ-1:** Identify gaps in evidence.
- **RQ-3:** Identify specific populations and health decision(s) affected by the research.
- **RQ-5:** Select appropriate interventions and comparators.
- **RQ-6:** Measure outcomes that people representing the population of interest notice and care about.

**List is not exhaustive*

Methodology Standards: Design & Analysis (1/2) *

- **RQ-2.** Develop a formal study protocol.

- **IR-1:** A priori, specify plans for quantitative data analysis that correspond to major aims.
- **IR-2:** Assess data source adequacy.
- **IR-5:** Provide sufficient information in reports to allow for assessments of the study's internal and external validity.

- **MD-2:** Use valid statistical methods to deal with missing data that properly account for statistical uncertainty due to missingness.
- **MD-4:** Examine sensitivity of inferences to missing data methods and assumptions, and incorporate into interpretation

Methodology Standards: Design & Analysis (2/2) *

- **CI-I:** Specify the causal model underlying the research question *(cross-cutting standard, applies to all PCOR/CER studies).*
- **CI-2:** Define and appropriately characterize the analysis population used to generate effect estimates
- **CI-3:** Define with the appropriate precision the timing of the outcome assessment relative to the initiation and duration of exposure.
- **CI-4:** Measure potential confounders before start of exposure and report data on potential confounders with study results.

**List is not exhaustive*

Increasing Value in Research: Challenges in the PCORI Portfolio (1/2)

- **Emphasis on Patient-Reported Outcomes (PROs)**
  - Requirements for contact with study participants for data collection
    - Approaches to data collection (phone banks, web-based portals)
    - Problems with missing data
  - PROs are not always the most relevant patient-centered outcomes

- **Study Designs**
  - Choosing "trendy" study design (cluster and SMART designs) vs. most appropriate design
  - Simplified consent processes

- **Delivery of interventions**
  - Compliance with treatment assignment (cross-over)
  - Fidelity to intervention
  - Expensive interventions

Increasing Value in Research: Challenges in the PCORI Portfolio (2/2)

- **Data Quality**
  - Use of electronic health record (EHR) data in CER for cohort identification and outcome assessment
    - Completeness, accuracy, and consistency

- **Analysis Plans**
  - Unrealistic (and unsupported) estimates of effect size
  - Focus on confounding as the only potential source of bias
    - No systematic approach to addressing potential confounding
  - Inappropriate use of heterogeneity of treatment effect (HTE) analyses

Discussion: Efforts to Address Scientific Challenges in PCORI's Portfolio

- What issues should be the priority for ongoing guidance and/or enforcement?

- To what extent can methodological issues be addressed by improved guidance, including additional (or revised) Methodology Standards?

LUNCH

11:30 AM – 12:30 PM

Recognition of Panel Members Completing Terms as of Spring 2018 CEDS Advisory Panel Meeting

Panel Member	Stakeholder Group
Michael Herndon	Payers
Leslie Levine	Patients, Caregivers, and Patient Advocates
Roy Poses	Researchers
Robert Bonomo	Clinicians
Jonathan Klein	Researchers
Kristin Voorhees	Patients, Caregivers, and Patient Advocates

Thank you for your contributions!

Orientation to Small Group Discussions

David Hickam, MD, MPH

Program Director
Clinical Effectiveness and Decision Science

Setting CEDS Future Research Priorities

- Following the CDR and APDTO portfolio updates presented this morning, and in consideration of the methodological issues discussed, PCORI is seeking input from the CEDS Advisory Panel to help set our future research priorities.
- Three small groups – note the color of the sticker on your table tent.
- Facilitators:
  - Leslie Levine (Yellow Group | *Constitution Ballroom E/D/C*)
  - Jeff Hersh (Blue Group | *Wilson*)
  - Nancy Perrin (Green Group | *Roosevelt*)
- Groups will reconvene at 2:10 pm to report back and identify common themes and priorities

Questions for the CEDS Advisory Panel

1. Based on the themes of the portfolios of funded projects, what are the priorities for future directions?
   a. Areas in need of further growth
   b. Important gaps
   c. Emerging trends in American health care

2. Are there areas of overlap between the two portfolios that should be considered and/or combined when thinking about future priorities?

Questions for the CEDS Advisory Panel

3. What future research priorities or areas of focus would address lessons learned?
   a. Methodological challenges
   b. Overall value of our funded research

4. Are there other types of portfolio analysis that would be useful in helping us set future research priorities?

BREAK

Wrap up, Next Steps, Debrief

ADJOURN