

Welcome!

**Please be seated by 8:20 am ET
The teleconference will go live at 8:30 am ET**



Communication and Dissemination Research

Advisory Panel Meeting

November 3, 2017

8:30 AM – 3:30 PM



PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Housekeeping

- Today's webinar is **open to the public** and is being **recorded**
- Members of the public are invited to listen to this teleconference and view the webinar
- Meeting materials can be found on the PCORI website
- Comments may be submitted through the webinar chat function, or by e-mail to advisorypanels@pcori.org
- For those in the room, please remember to speak loudly and clearly **into the microphone**. State your **name** and **affiliation** when you speak.
- Where possible, we encourage you to avoid technical language.
- Visit www.pcori.org/events for more information



Agenda

- 8:30 AM - Welcome and Introductions/ Program Updates
- 9:00 AM – Update on CDR Framework Research Paper
- 9:20 - Uncertainty Small Group Breakout Session
- 10:20 – Break
- 10:30 – Dissemination Small Group Breakout Session
- 11:30 – LUNCH
- 12:30 – History of CDR/APDTO Advisory Panels
- 1:00 – PCORI Science
- 1:30 – Current State of Affairs
- 2:15 – Pragmatic Clinical Studies Prioritization Topics
- 3:30 Adjourn



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.



Program Updates

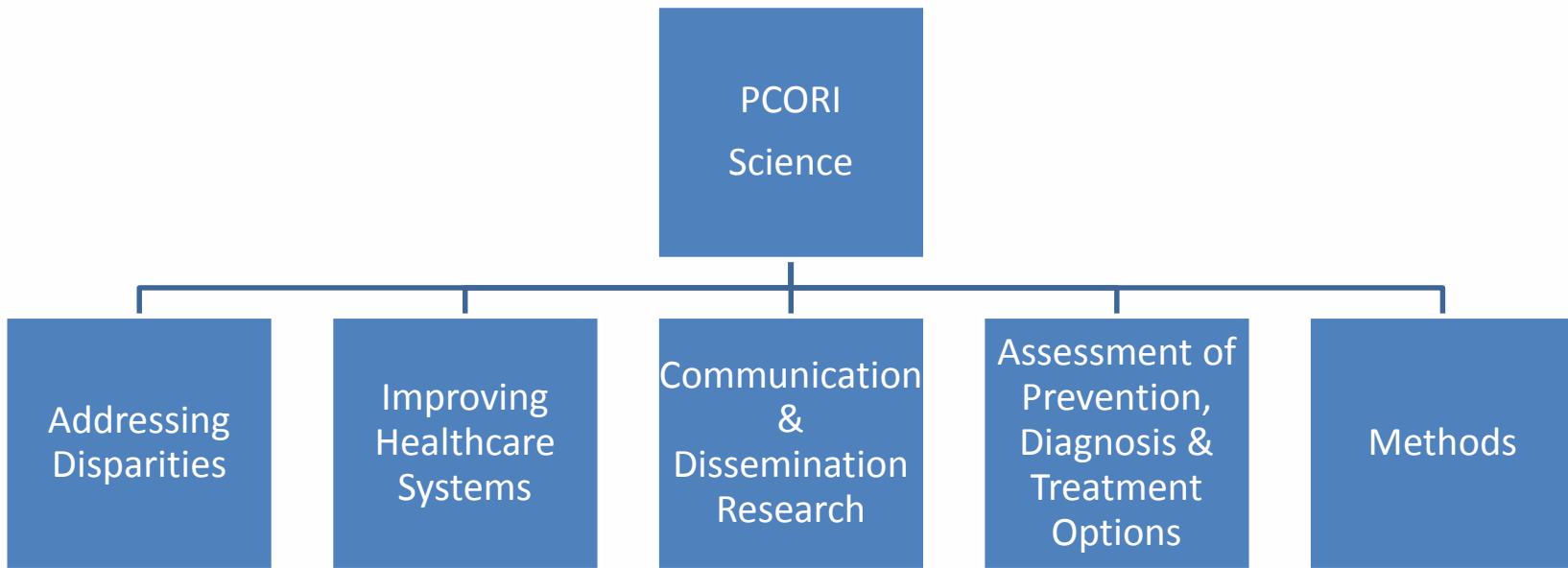
William Lawrence, MD, MS

Associate Director, Clinical Effectiveness and Decision Science

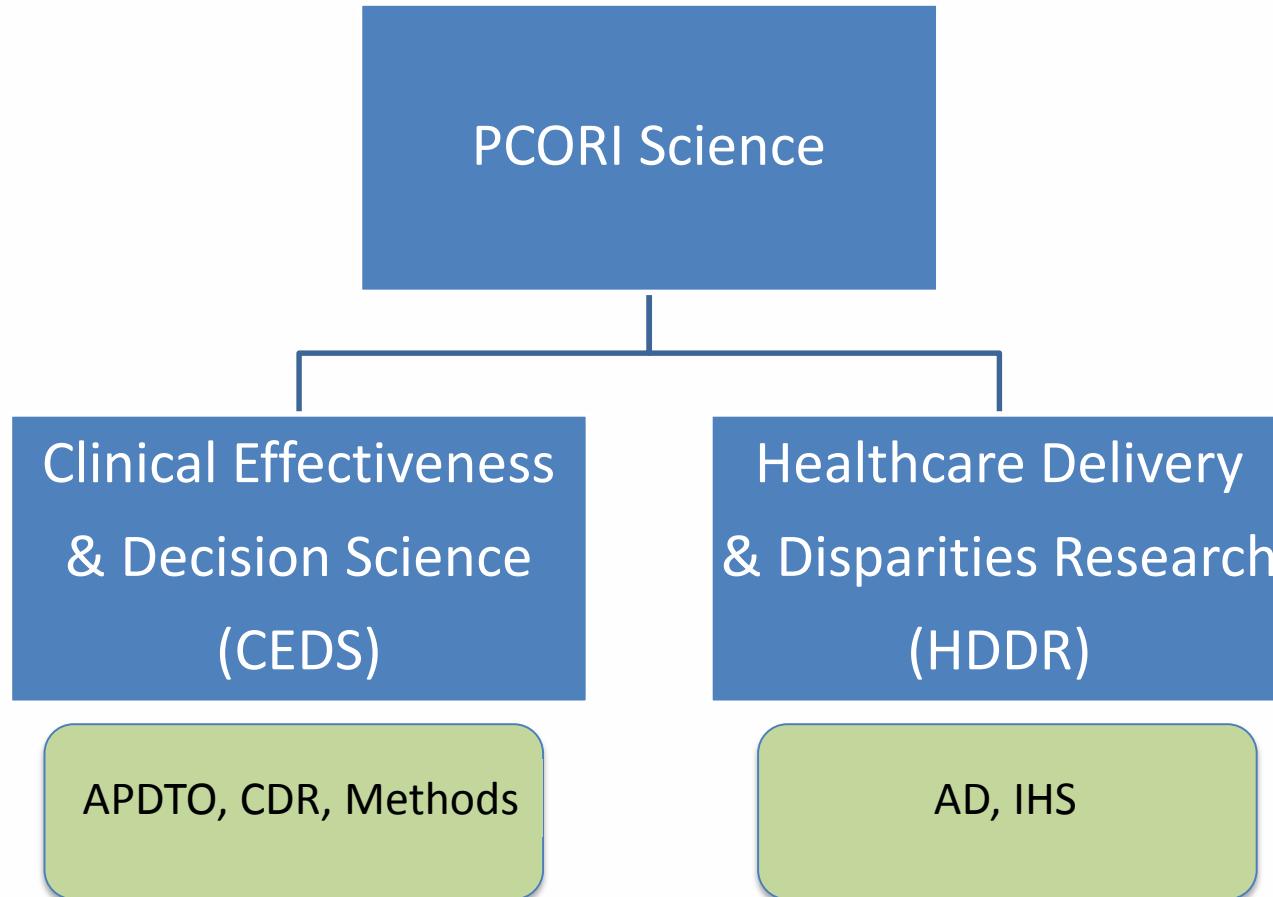


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Science (Pre-re-organization)



Current Science Organization



CDR Research Priority

- Broad Awards – 47 funded projects
 - 41 Communication strategies
 - 7 Explaining uncertainty
 - 6 Dissemination strategies
- New Broad Award - Engaging Parents of Children with Sickle Cell Anemia and their Providers in Shared-Decision Making for Hydroxyurea



Targeted Funding Announcements

- Community-Based Palliative Care Delivery for Adult Patients with Advanced Illness and their Caregivers
 - Population-Based Comparison of Evidence-Based, Patient-Centered Advance Care Planning Interventions on Advance Directive Completion, Goal Concordant Care and Caregiver Outcomes for Patients with Advanced Illness
 - Reducing Disparities in the Quality of Palliative Care for Older African Americans through Improved Advance Care Planning (EQUAL ACP)
 - A Cluster-Randomized Trial Comparing Team-Based versus Primary Care Clinician-Focused Advance Care Planning in Practice-Based Research Networks



Targeted Funding Announcements (2)

- Strategies to Prevent Unsafe Opioid Prescribing in Primary Care among Patients with Acute or Chronic Non-Cancer Pain
 - What is the 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?



Questions/Comments?

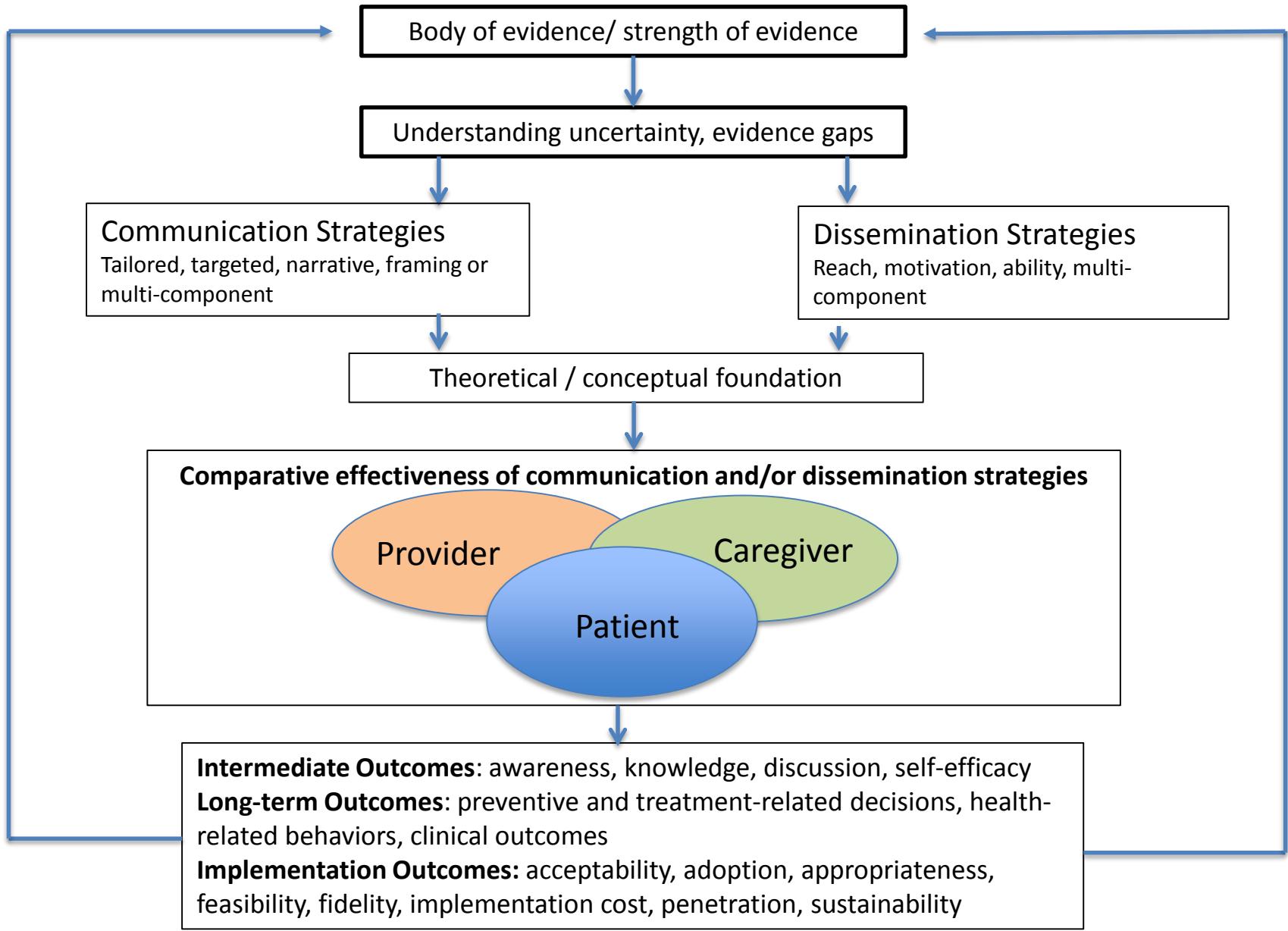


Communication and Dissemination Research (CDR) Framework Paper Update

Bridget Gaglio, PhD, MPH

Senior Program Officer, Clinical Effectiveness and Decision Science





Uncertainty Breakout Session

November 3, 2017

9:20-10:20 AM



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CDR Current Priorities

- Communication strategies to promote the use of health and health care CER evidence by patients and clinicians;
- Dissemination strategies to promote the use of health and health care CER evidence by patients and clinicians;
- Explaining uncertain health and health care CER evidence to patients and clinicians.



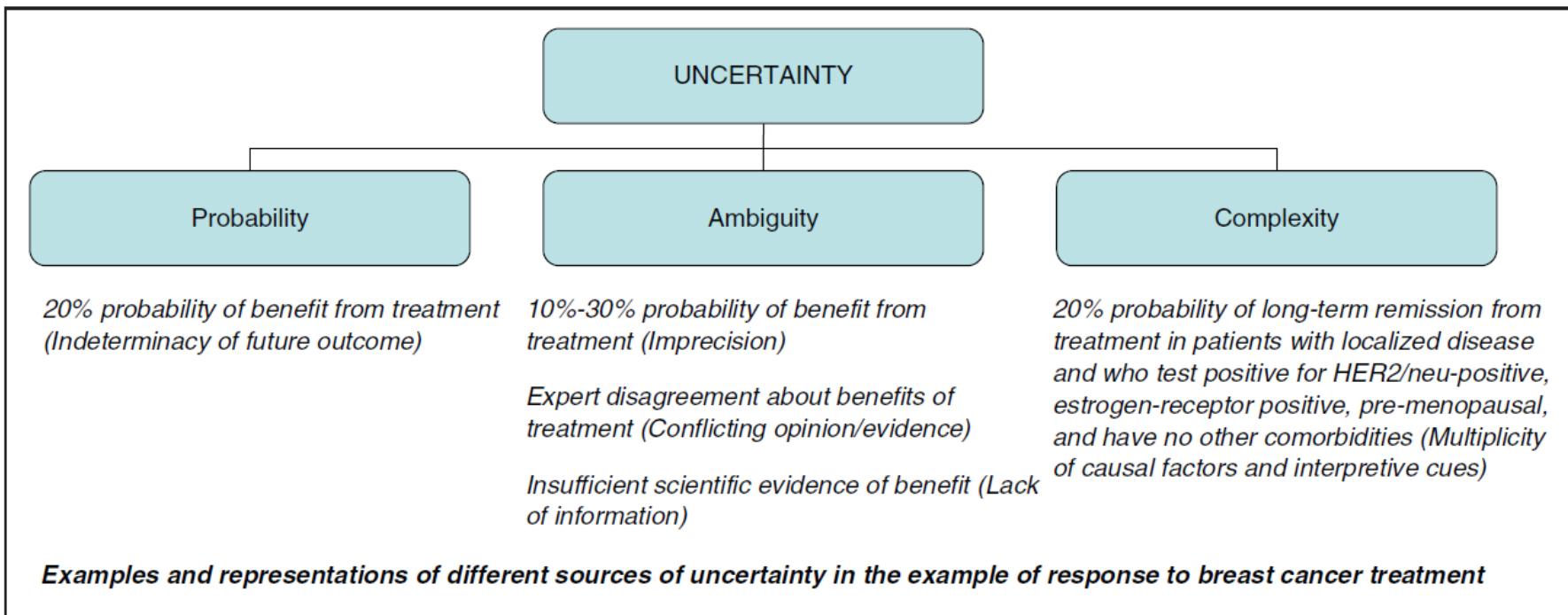


Figure 1. Sources of uncertainty in health care.

Han PKJ, et al. *Med Decis Making* 2011;31:828.



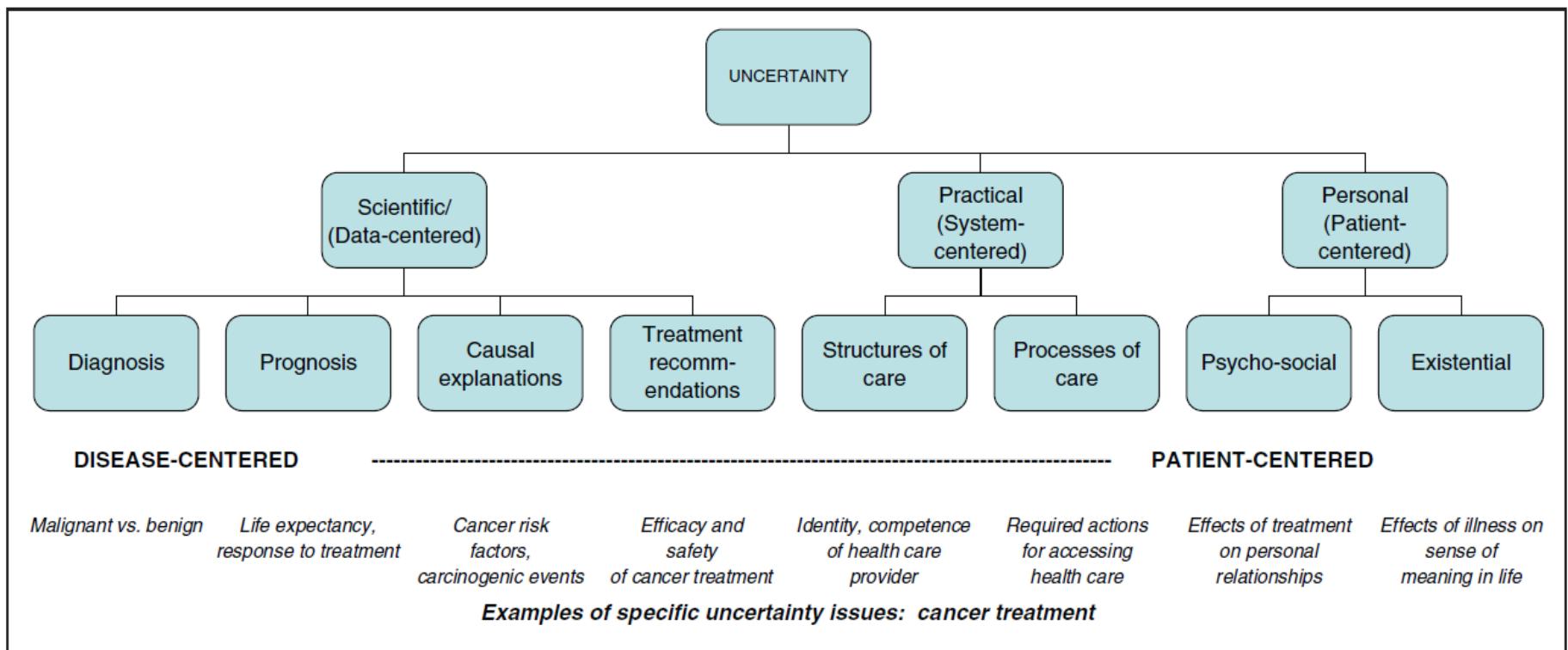


Figure 2. Issues of uncertainty in health care.

Han PKJ, et al. *Med Decis Making* 2011;31:828.



Questions

- How has uncertainty influenced your experience as a patient or clinician? What impact did it have and why was that the case?
- What was the most challenging aspect of communicating about uncertainty?
- Given uncertainty in evidence will continue to exist, what do you think are the most important ways to communicate about/explain it to promote evidence-based decisions?



BREAK

10:20 am – 10:30 am



Dissemination Breakout Session

November 3, 2017

10:30 am - 11:30 am



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CDR Current Priorities

- Communication strategies to promote the use of health and health care CER evidence by patients and clinicians;
- Dissemination strategies to promote the use of health and health care CER evidence by patients and clinicians;
- Explaining uncertain health and health care CER evidence to patients and clinicians.



What is dissemination?

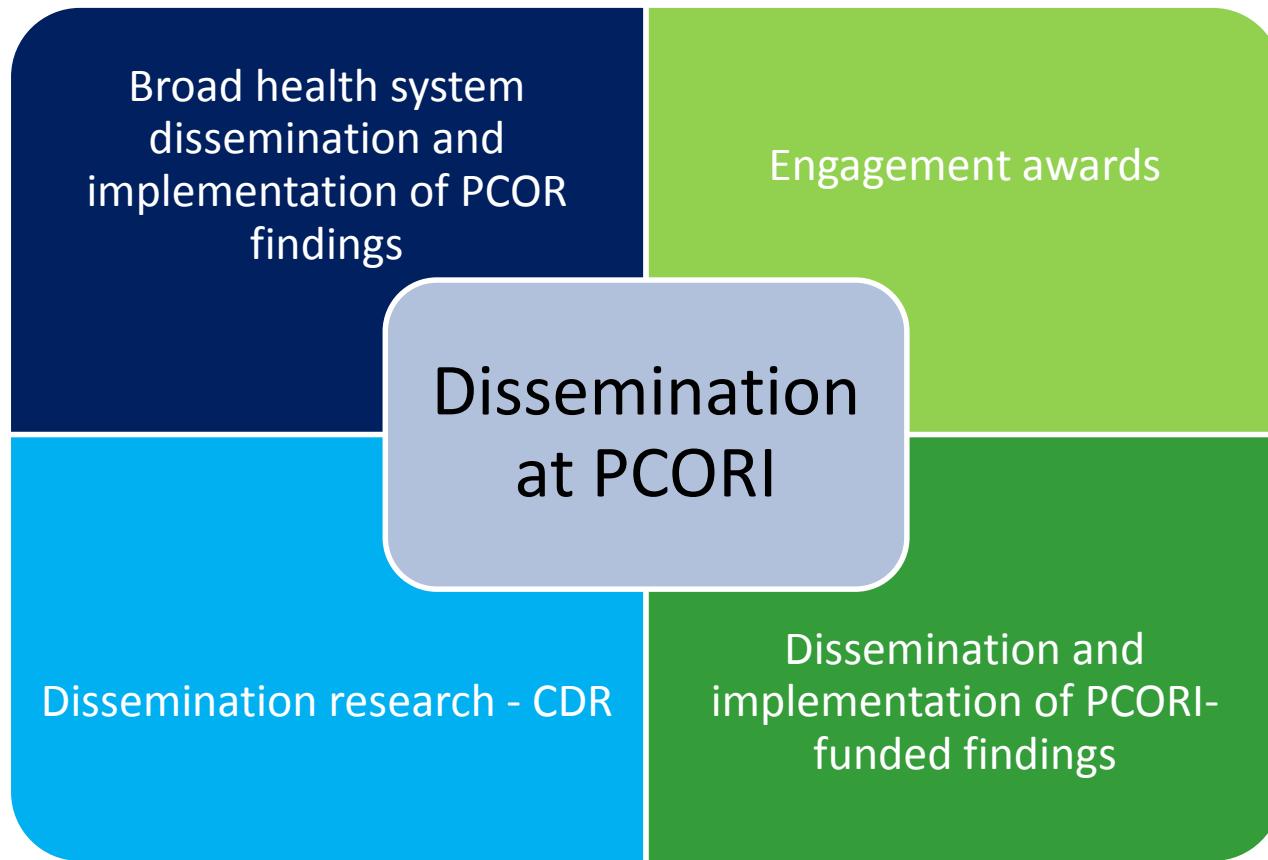
- *Dissemination* is defined as.....
 - ***the active and targeted*** approach of spreading evidence-based interventions to potential adopters and the target audience through determined channels using planned strategies.

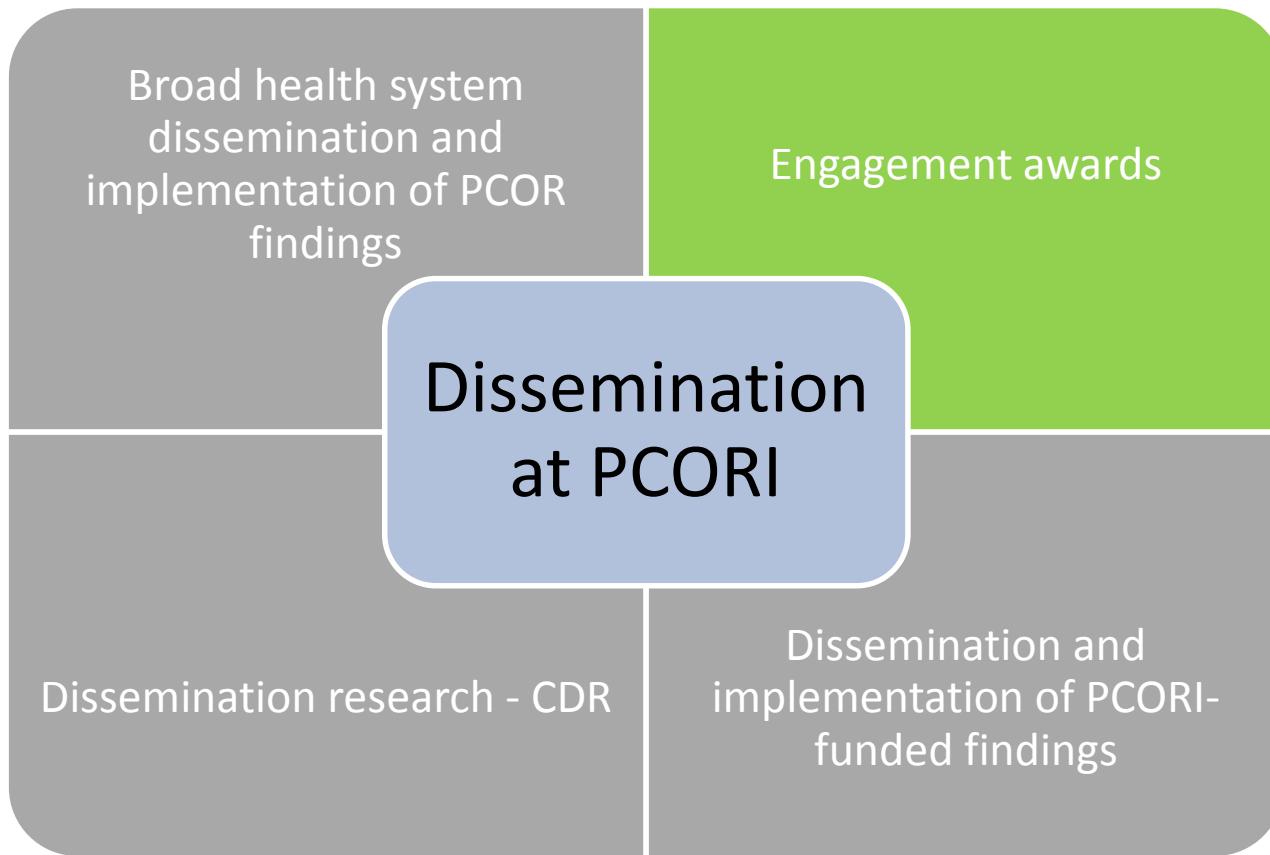


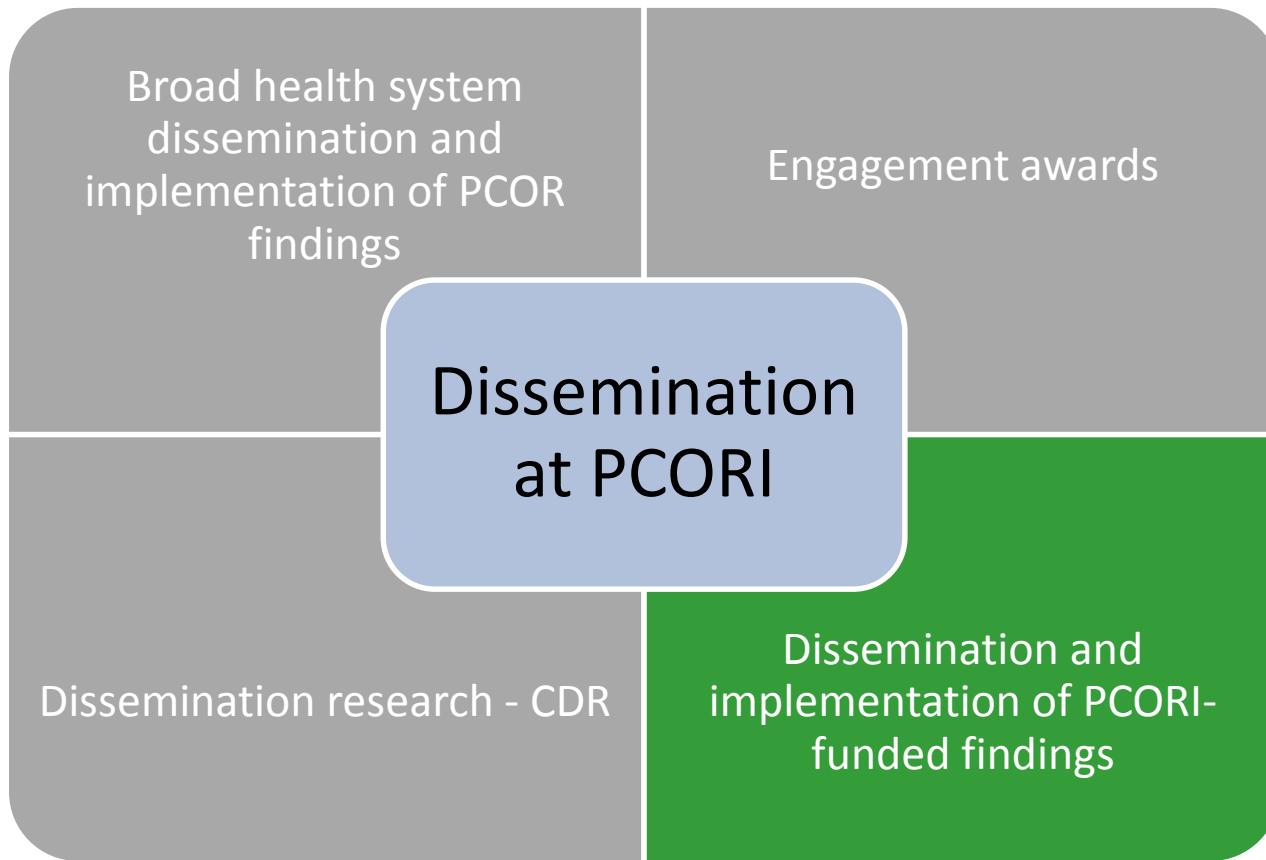
What is implementation?

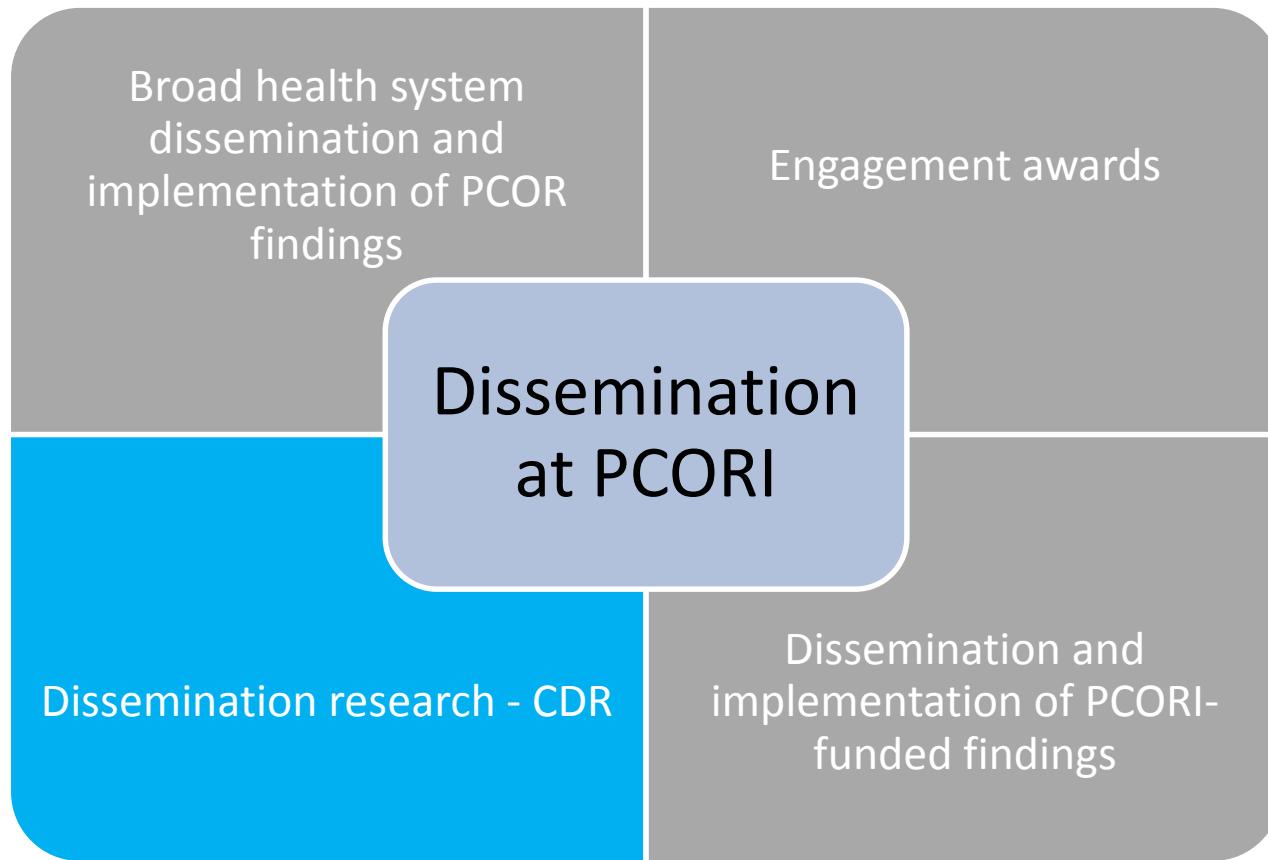
- *Implementation* is the.....
 - **deliberate**, iterative process of integrating evidence into policy and practice through adapting evidence to different contexts and facilitating behavior change and decision making based on evidence across individuals, communities, and healthcare systems.

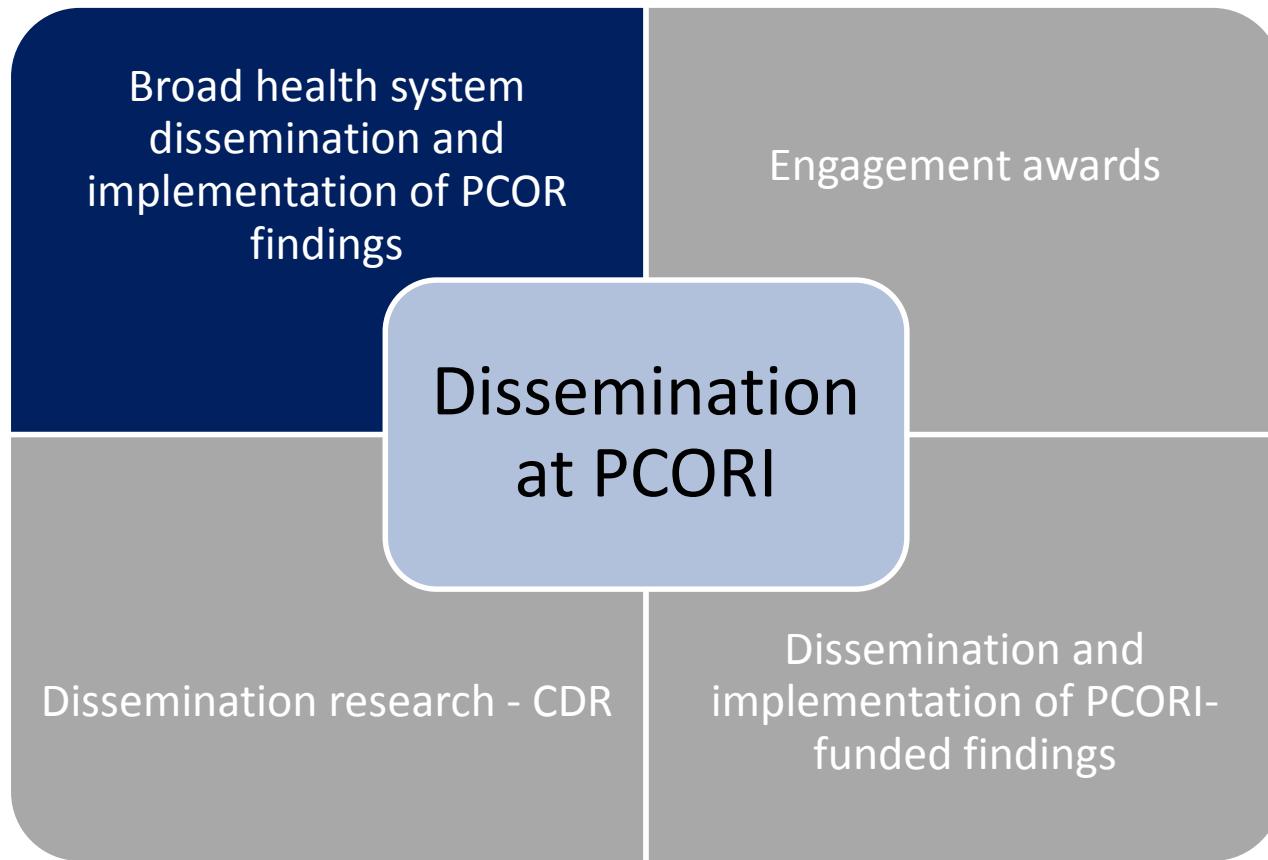












Questions

- What is your experience with dissemination science?
- We have been focused on identifying the most effective approaches to disseminating CER results to healthcare providers, with the goals of sustained changes in clinical practice and effective dissemination to patients of results that enable behavior change. Are there other areas in the field we should be focused on?
- Are there evidence gaps around these other areas of potential emphasis?
Such as:
 - In the audiences studied
 - Methods used; e.g. as social media analysis
 - Clinical areas studied
 - The role of stakeholders and social networks in the dissemination process



LUNCH

11:30 am – 12:30 pm



History of CDR / APDTO Advisory Panel

David Hickam, MD, MPH

Program Director, Clinical Effectiveness and Decision Science, PCORI

Stanley Ip, MD

Associate Director, Clinical Effectiveness and Decision Science, PCORI

William Lawrence, MD, MS

Associate Director, Clinical Effectiveness and Decision Science, PCORI



Joint CDR / APDTO Panel Meeting

Context and Objectives

- The 2016 Science reorganization reflects PCORI's vision of how to align our national research priorities with programmatic functions and structure
 - Clinical Effectiveness and Decision Science
 - Healthcare Delivery and Disparities Research
- The PCORI Board of Governors will review the activities of the Advisory Panels
 - Refocusing of programmatic Advisory Panels
- Today's afternoon session provides opportunity for CDR / APDTO panels to meet jointly, learn the history of both panels, and engage in collaborative discussion



History of CDR Priority

- Communication and Dissemination Research established as one of 5 National Research Priorities in 2012
 - “Comparing approaches to providing comparative effectiveness research information, empowering people to ask for and use the information, and supporting shared decision-making between patients and their providers.”
- Original PFA for the CDR Priority issued in 2012
- CDR Advisory Panel Charter approved in 2015



CDR Funding

- Focus of the current PFA:
 - Communication strategies to promote the use of health and health care CER evidence by patients and clinicians;
 - Dissemination strategies to promote the use of health and health care CER evidence by patients and clinicians;
 - Explaining uncertain health and health care CER evidence to patients and clinicians.
- Currently, total of 47 projects funded under the CDR Priority
 - 41 Communication
 - 6 Dissemination
 - 7 Explaining Uncertainty (also have a communication component)



History of APDTO Priority

- Assessment of Prevention, Diagnosis, and Treatment Options (APDTO) established as one of 5 National Research Priorities in 2012
 - “Comparing the effectiveness and safety of alternative prevention, diagnosis, and treatment options to see which ones work best for different people with a particular health problem.”
 - 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
- Original PFA for the APDTO Priority issued in 2012
- Awarded 118 projects through Cycle 3 2016



History of APDTO Advisory Panel

- APDTO Advisory Panel first met in April 2013
- Today's meeting is the 15th meeting of the APDTO panel
- 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 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



Questions / Discussion



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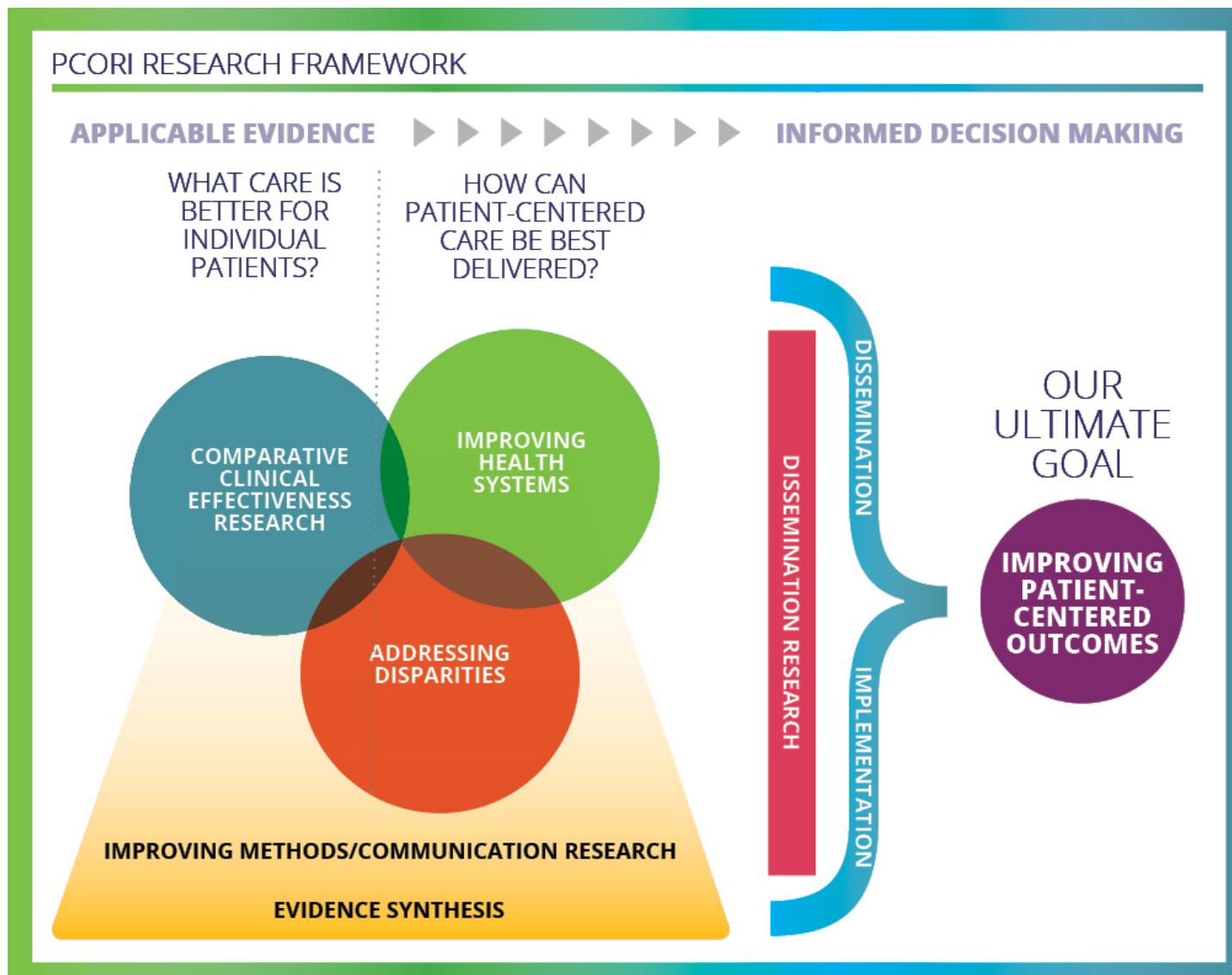
PCORI Science

Evelyn P. Whitlock, MD, MPH

Chief Science Officer



Our Research Framework



Research and Evidence Synthesis



PCORI and Evidence Synthesis

- PCORI's authorizing legislation states that **evidence synthesis** is a core function of PCORI:

“(C) PURPOSE.—The purpose of the Institute is to assist patients, clinicians, purchasers, and policy-makers in making informed health decisions by advancing the quality and relevance of evidence concerning the manner in which diseases, disorders, and other health conditions can effectively and appropriately be prevented, diagnosed, treated, monitored, and managed through research and evidence synthesis that considers variations in patient subpopulations....”



Evidence Synthesis

- Methodologies for integrating evidence from variable sources to produce more comprehensive or best evidence
 - Provides knowledge beyond individual studies alone
 - Identifies areas of agreement and disagreement in quantitative and/or qualitative terms
 - Permits identification of research gaps
 - Examples: **Systematic reviews**, rapid reviews, decision models, analytic approaches (e.g., aggregate data meta-analysis (MA), individual patient-level data (IPD) MA, network MA, others)



PCORI's Research Synthesis Program (2017)

- Three initial goals:
 1. Research to address heterogeneity of treatment effects, more personalized individual health care choices
 2. More rapid deployment of actionable CER evidence in context
 3. Communication of current portfolio (rationale, themes and lessons, context)



Areas of Portfolio Focus

Our website highlights additional PCORI Research Areas
www.pcori.org/research-results/research-topics

Cardiovascular Disease

Learn about our funded research on heart disease, the leading cause of death nationally.



Cancer

Read about our portfolio addressing cancer, the no. 2 cause of death in the United States.



Pain Care and Opioids

Read about our funded projects on managing chronic pain and addressing opioid use.



Kidney Disease

Read about our funded research on which treatments work best for patients.



Multiple Sclerosis

Learn about the research we're funding to help improve the lives of Americans with MS.



Dementia and Cognitive Impairment

Read about our funded studies on dementia and cognitive impairment, including Alzheimer's disease.



Transitional Care

Learn about projects on improving transitions between healthcare settings or providers.



Questions?

Evelyn P. Whitlock, MD, MPH

Chief Science Officer



PCORI CDR/ADPTO Advisory Panel: Public Policy Update

Andrew Hu

Director, Public Policy and Government Relations

Jean Slutsky

Chief Engagement and Dissemination Officer



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THE US LEGISLATIVE PROCESS

SENATE

A BILL IS INTRODUCED BY A MEMBER OF THE SENATE AND ASSIGNED TO A COMMITTEE FOR REVIEW.

THE COMMITTEE MEETS TO DISCUSS, AMEND, AND VOTE ON THE BILL.



IF APPROVED, BILL PROCEEDS TO THE FULL SENATE FOR FURTHER DISCUSSION, AMENDMENTS, AND VOTING.



PRESIDENT SIGNS THE BILL AND IT BECOMES A LAW.

IT STARTS WITH AN IDEA...



BILLS MUST PASS THROUGH BOTH CHAMBERS BEFORE BEING SENT TO THE PRESIDENT.

HOUSE OF REPRESENTATIVES

A BILL IS INTRODUCED BY A MEMBER OF THE HOUSE AND ASSIGNED TO A COMMITTEE FOR REVIEW.

THE COMMITTEE MEETS TO DISCUSS, AMEND, AND VOTE ON THE BILL.



IF APPROVED, BILL PROCEEDS TO THE FULL HOUSE FOR FURTHER DISCUSSION, AMENDMENTS, AND VOTING.

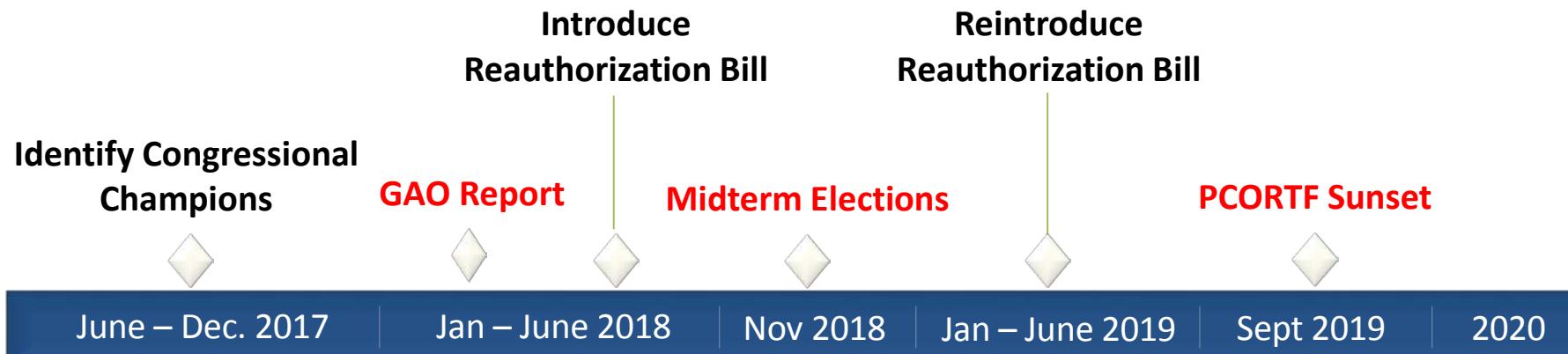


THE VETO CAN BE OVERRIDDEN BY CONGRESS WITH A 2/3 VOTE.

PRESIDENT VETOES THE BILL AND SENDS IT BACK TO CONGRESS.



Timeline for Reauthorization



As we work to reauthorize PCORI's funding for the future, it is important to know that PCORI is committed to fulfilling our mandate from Congress and will continue to exist and support the generation of patient-centered research beyond 2019.



Current Priorities for Reauthorization

- Increase awareness of PCORI to policymaking community
- Showcase the value and impact of research
- Continued engagement with key stakeholders
- Frame the role of PCORI for the next 10 years
- Build upon third-party validation



What PCORI is Doing

Education and Increasing Awareness	Highlighting Results and Potential Impacts	Identifying Potential Policy Roles	Building and Mobilizing Third-Party Support
<ul style="list-style-type: none">• Direct engagement with Congressional staff and policymakers• Congressional briefings• Thought-leadership activities• Increased media presence	<ul style="list-style-type: none">• Promoting final results• Developing economic impact analysis of study findings (<i>loss of work, decreased hospitalizations, etc.</i>)	<ul style="list-style-type: none">• Early access to FDA data to support drug pricing/value debate• Real-world evidence and early-market surveillance activities• Coverage with evidence development• Role in identifying key patient-reported outcomes	<ul style="list-style-type: none">• Identify and leverage third-party validators• Activating PCORI validators and direct engagement with patient and stakeholder organizations• Managing key stakeholder perspectives and opinions



Examples of Patient Org Engagement

- Targeting 40+ patient stakeholders for personal touches

Recent Meetings

- ✓ American Diabetes Association
- ✓ American Heart Association
- ✓ American Lung Association
- ✓ Lung Cancer Alliance
- ✓ National Organization of Rare Disorders

Key Requests

- More frequent updates throughout lifecycle of relevant projects
- Increase awareness of results timeline
- More accessible view of PCORI portfolio, e.g. where projects intersect with subtopics and populations of interest
- Interest in economic modeling

PCORI Follow-up

- Provided more detailed updates on specific studies of interest, per PCORI Program Officers.
- Created portfolio crosswalks based on subtopic, population, and types of outcomes.
- Arranged a PCORI speaker at NORD Annual Meeting.
- Opened door for ongoing dialogue.



Examples of Stakeholder Org Engagement

- Strategic targeting of medical specialty societies

Recent Meetings

- ✓ American College of Surgeons
- ✓ American Medical Association
- ✓ Society of Thoracic Surgeons
- ✓ American Association of Neurological Surgeons

Key Requests

- Improve review process to make it easier for societies to apply for research
- Fund studies that utilize physicians registries
- Interested in further engagement regarding implementation and implementation strategies
- More accessible understanding of our portfolio

PCORI Follow-up

- Planning a medical specialty society roundtable for Jan. 2018
- Working with individual societies to support research topic generation
- Engaging specialty societies around dissemination and implementation opportunities
- Leverage BoG relationships to increase PCORI engagement and presence at society meetings



Meeting

Example of Congressional Briefing

PCORI and Anthem cohosted a briefing on the need for evidence-based strategies to address America's opioid epidemic. Speakers included **Senator Shelly Moore Capito** (R- WV).



Stakeholders

Anthem (cohost), PCORI-funded researcher (Erin Krebs, MD, MPH), Veterans Health Administration researcher (Stephanie Tayler, PhD, MPH), patient partner (Christine Veasley)



PCORI will continue to use its **convening power** to demonstrate the crucial role **clinical comparative effectiveness research** will play in both solving the immediate opioid crisis, and building an **evidence base** for alternative chronic pain treatment options.

Importance



Examples of Third-Party Validation



The Value of Comparative Effectiveness Research

Dr. Phil Gingrey, The District Policy Group
([link](#))

“ *...I am impressed with the PCORI studies I reviewed and am encouraged by the manner in which the information is being disseminated and leveraged – to the benefit of patients, physicians and the health care system, and not in an autocratic way.*

The Future of Comparative Effectiveness Research

Hannah Martin, Bipartisan Policy Center ([link](#))

“ *...PCORI enjoys broad bipartisan support for its mission to provide providers with the best evidence-based information on treatments, while also giving them the flexibility to tailor treatments to each individual patient.*



Prioritization of PCS Topics

**Assessment of Prevention Diagnosis and Treatment Options and
Communication and Dissemination Research Joint Advisory Panel
Meeting**

November 3, 2017

David Hickam, MD MPH

Program Director, Clinical Effectiveness and Decision Science, PCORI

Stanley Ip, MD

Associate Director, Clinical Effectiveness and Decision Science, PCORI

Rebecca Barasky, MPH

Program Manager, Clinical Effectiveness and Decision Science, PCORI

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Goals for PCS Topics Session

- Review PCORI's priority topics relevant to the Clinical Effectiveness and Decision Science Program (APDTO panel has previously reviewed all but insomnia):
 - ✓ Community-acquired pneumonia
 - ✓ Treatment strategies for symptomatic osteoarthritis (OA), including joint replacement
 - ✓ Surgical options for hip fracture in the elderly
 - ✓ Studies of patients with non-muscle invasive bladder cancer (NMIBC) who failed first-line treatments
 - ✓ Treatments for insomnia
- Revisit topics to obtain Advisory Panelists' input on PCORI's investment in future funding initiatives – do any warrant special emphasis, larger investments, or targeted funding announcements?
- Prioritize topics by importance and alignment with PCORI's Research Criteria



PCORI Tier 3 Research Criteria

- **Patient-Centeredness:** is the comparison relevant to patients, their caregivers, clinicians, or other key stakeholders and are the outcomes relevant to patients?
- **Impact of the Condition on the Health of Individuals and Populations:** Is the condition or disease associated with a significant burden in the U.S. population, in terms of disease prevalence, costs to society, loss of productivity or individual suffering?
- **Assessment of Current Options:** Does the topic reflect an important evidence gap related to current options that is not being addressed by ongoing research?
- **Likelihood of Implementation in Practice:** Would new information generated by research be likely to have an impact in practice? (E.g., do one or more major stakeholder groups endorse the question?)
- **Durability of Information:** Would new information on this topic remain current for several years, or would it be rendered obsolete quickly by new technologies or subsequent studies?



Pragmatic Clinical Studies

Background and Purpose

- Program launched in early 2014 to expand support of high-priority patient-centered comparative clinical effectiveness research
- Program's purpose is to fund large pragmatic clinical trials, large simple trials, or large-scale observational studies that compare two or more meaningful clinical alternatives (including complex interventions)
- Initiative emphasizes that we seek pragmatic studies appropriate for a specific high-priority question
- High-priority research questions may come from several sources:
 - IOM's Priorities for CER
 - AHRQ's Future Research Needs Projects
 - Topics recommended by patients and stakeholders through PCORI's topic prioritization process (PCORI Priority Topics)



Community Acquired Pneumonia:

History of Topic and Funding

- **Nominator / Topic Source:** American College of Physicians -Clinical Guidelines Committee
- **Reviewed by APDTO Advisory Panel:** May 2015
- **Added to PCS Priority List:** Cycle 2, 2016 funding announcement
- **Current PCS Priority List Question:** What is the comparative effectiveness and safety of alternative FDA-approved antibiotic regimens in the empiric outpatient treatment of adults with community-acquired pneumonia?
- **PCORI Funding:**
 - No studies funded under this priority topic to date
 - Received a few applications in PCS Cycle 2, 2017 - currently under review



Community Acquired Pneumonia:

Topic Brief Summary

- **Prevalence:**
 - In 2012, 1.1 million persons were diagnosed with CAP
 - Estimated 915,900 episodes of CAP occur in adults 65+ each year in the U.S.
- **Available Treatment Options:**
 - Antibiotics for CAP caused by bacteria: Narrow-spectrum recommended for young patients; broad-spectrum used in older patients or those with comorbidities and/or severe disease
- **Decisional Dilemma**
 - Questions remain about the usefulness of diagnostic tests and their impact on patient-centered outcomes, as well as regarding the selection of narrow vs broad-spectrum antibiotics and the duration of treatment



Community Acquired Pneumonia:

Topic Brief Summary

- **Patient Centered Outcomes of Interest:**
 - Hospital and ICU admission rate, length of stay and readmission rate
 - Short-term disability; days away from work/school/normal activities; lost productivity
 - Cost of care
 - Patient satisfaction: emergence of resistance, infection
 - Drug toxicity; adverse events; mortality
- **Evidence Gaps / Research Areas of Interest:**
 - Comparative effectiveness (CE) of alternative approaches to treating CAP (broad vs narrow-spectrum for empiric and/or definitive therapy) – variable comparisons in RCTs have limited ability to pool data
 - CE of new techniques to determine pathogens and establish diagnosis to choose the most appropriate antibiotic regimens or avoid them when unnecessary
 - CE of shorter vs longer antibiotic therapy and approaches to de-escalate antibiotic therapy



Osteoarthritis:

History of Topic and Funding

- **Nominator / Topic Source:** Institute of Medicine
- **Reviewed by APDTO Advisory Panel:** April 2013
- **Added to PCS Priority List:** Spring 2014 funding announcement
- **Current PCS Priority List Question:** Compare the effectiveness of treatment strategies for symptomatic osteoarthritis (OA) including joint replacement
- **PCORI Funding:**
 - No PCS projects funded under this priority topic to date
 - PCORI has funded several smaller projects focusing on osteoarthritis through the Broad PFAs



Osteoarthritis:

Topic Brief Summary

- **Prevalence:**
 - 27 million US adults (>10% of population) aged 18 years and older have one or more type of clinical OA. Prevalence varies by definition of OA, location of OA, and populations studied
- **Available Treatment Options:**
 - Pain relievers and nonsteroidal anti-inflammatory drugs
 - Exercise and physical therapy; weight loss
 - Combination management
 - Joint surgery
- **Decisional Dilemma**
 - Given the high burden of disease and impact on patient-centered outcomes what management strategy (or combination) works best for key subgroups?
 - What are the comparative benefits/harms of different management strategies and which are effective in fostering long-term adherence in real-world clinical settings?



Osteoarthritis:

Topic Brief Summary

- **Patient Centered Outcomes of Interest:**
 - Quality of life
 - Productivity
 - Functional capacity
 - Mortality
- **Evidence Gaps / Research Areas of Interest**
 - There are few comparative effectiveness studies of exercise and physical therapy strategies or multimodal treatments; understanding the best interventions in this area could improve care and outcomes by establishing a set of “best practices”
 - Methods for implementing and sustaining effective treatment strategies in real-world clinical settings are lacking; particularly for non-medication based strategies
 - Comparative effectiveness research is needed to determine which key subgroups of patients do best with a given management strategy



Hip Fracture:

History of Topic and Funding

- **Nominator / Topic Source:** Agency for Healthcare Research and Quality
- **Reviewed by APDTO Advisory Panel:** August 2014
- **Added to PCS Priority List:** Winter 2015 funding announcement
- **Current PCS Priority List Question:** Compare the effectiveness of different surgical treatments in elderly patients with hip fractures in terms of functionality and other patient-centered outcomes
- **PCORI Funding:**
 - One related PCS project funded: “A Practical Intervention to Improve Patient-Centered Outcomes after Hip Fractures Among Older Adults”
 - Compares spinal vs general anesthesia on recovery of walking 60 days after surgery for hip fracture in adults 50+



Hip Fracture:

Topic Brief Summary

- **Incidence:**
 - 957 per 100,000 for women and 414 per 100,000 for men from 1986 to 2005
- **Available Treatment Options:**
 - Surgery: surgical treatment options vary widely by fracture type (e.g. hemi- or total arthroplasty, internal fixation, implants, etc.)
- **Decisional Dilemma**
 - Limited evidence exists to answer questions about the relationship between the selected surgical intervention or implant variables and patient outcomes, and between patient variables, fracture type, and patient outcomes
 - There remains a high degree of uncertainty as to the best way to treat unstable hip fractures and about which treatment options are best for various clinical populations



Hip Fracture:

Topic Brief Summary

- **Patient Centered Outcomes of Interest:**
 - Pain, quality of life
 - Functional capacity/impairment/independent living
 - Prolonged rehabilitation
 - Mortality
- **Evidence Gaps / Research Areas of Interest:**
 - Research is needed to identify predictors of short time-to-recovery and functional outcomes as well as the impact of suboptimal surgical quality on functional outcomes
 - Comparative effectiveness of optimal treatment strategies for different types of fractures or defined populations and between-class or within-class comparisons (e.g. nails vs screws, etc.)



Non-Muscle-Invasive Bladder Cancer:

History of Topic and Funding

- **Nominator / Topic Source:** American Urological Association
- **Reviewed by APDTO Advisory Panel:** November 2016
- **Added to PCS Priority List:** Cycle 1, 2017 funding announcement
- **Current PCS Priority List Question:** Compare the effectiveness of treatments in patients with intermediate or high-risk NMIBC who have failed first-line induction intravesical therapy with BCG or other agents
- **PCORI Funding:**
 - No studies funded under this priority topic to date



Non-Muscle-Invasive Bladder Cancer

Topic Brief Summary

- **Incidence:**

- Estimated 76,960 new cases of bladder cancer in the U.S. in 2016 (58,950 in men); 5% of all incident cancers in the U.S.

- **Available Treatment Options:**

- Main treatment is transurethral resection of bladder tumor (TURBT)
 - Adjuvant intravesical therapy: BCG; various chemotherapy agents or interferon immunotherapy
 - Radical cystectomy may be an option when there is high-risk of progression to muscle-invasive bladder cancer

- **Decisional Dilemma:**

- The best management of patients with intermediate- or high-risk NMIBC that have failed induction intravesical therapy with BCG remains uncertain. Head-to-head comparisons have shown few clear differences in outcomes, with moderate to low strength of evidence



Non-Muscle-Invasive Bladder Cancer:

Topic Brief Summary

- **Patient Centered Outcomes:**

- Mortality
- Need for cystectomy
- Progression to muscle-invasive bladder cancer
- Bladder cancer recurrence
- Quality of life

- **Evidence Gaps / Research Areas of Interest:**

- Comparative effectiveness of various intravesical agents, cystectomy or bladder-preserving alternatives to cystectomy, and/or novel agents on patient outcomes after failure of first-line therapy
- RCTs that compare initial cystectomy with intravesical therapy or other bladder-preserving therapies for high-risk NMIBC could provide information to inform treatment decisions
- Comparative effectiveness of approaches to reduce discomfort and/or adverse effects in patients



Insomnia:

History of Topic and Funding

- **Nominator / Topic Source:** PCORI
- **Reviewed by APDTO Advisory Panel:** N/A
- **Added to PCS Priority List:** Cycle 2, 2017 funding announcement
- **Current PCS Priority List Question:** Compare the benefits and harms of pharmacologic, psychological, or combination treatments for treating different types of insomnia on sleep and patient-centered outcomes including next-day function, mood, and quality of life
- **PCORI Funding:**
 - Topic added to most recent PCS funding announcement
 - Have not yet received applications addressing this priority topic



Insomnia:

Topic Brief Summary

- **Prevalence**
 - Approximately 1/3 of adults suffer from occasional symptoms of insomnia
 - Approximately 6% of adults experience chronic and persistent insomnia
- **Available Treatment Options**
 - Psychological Interventions: cognitive behavioral therapy (CBT-I); multicomponent behavioral/brief behavioral therapy (BBT)
 - Pharmacological Interventions: over the counter (sedating antihistamines, melatonin); prescription sleep aids
- **Decisional Dilemma**
 - Complexity of treatment choice due to number of options
 - Questions regarding risks and benefits of available options: few head-to head studies and quality of evidence ranges from insufficient to moderate



Insomnia:

Topic Brief Summary

- **Patient-Centered Outcomes of Interest**
 - Sleep outcomes
 - Next-day function, mood, quality of life
 - Adverse effects of treatments
- **Evidence Gaps / Research Areas of Interest:**
 - Comparative effectiveness of various psychological and pharmacological treatment options
 - Long-term safety and effectiveness of pharmacological options (follow-up over one year)
 - Head-to-head comparisons of alternative methods for delivering CBT-I given limited availability of providers
 - Trials that include baseline data on sleep outcomes and patient-reported mood, quality of life outcomes



Ranking and Prioritization of Topics

- Based on a review of the topics and their alignment with PCORI's research criteria, how would you rank the topics in order of importance?
- What are PCORI's next steps for funding initiatives and investments in each topic?
- Do any of the topics rise to the level of a special emphasis question or targeted PFA?



Wrap Up

- Next in-person meeting Spring 2018
- Questions/Comments?



Thank you for your participation

**Advisory Panel on Assessment of Prevention,
Diagnosis, and Treatment Options**

**Advisory Panel on Communication and
Dissemination Research**

November 3, 2017

