Patient Engagement Advisory Panel
Spring 2016 Meeting

April 11, 2016

Washington, DC

pcori

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Agenda

8:30 a.m.	Welcome and Review Agenda
8:45 a.m.	New Staff Introductions
9:00 a.m.	Q&A with Joe Selby
9:45 a.m.	Patient Engagement in Action
11:00 a.m.	Telling the PCORI Engagement Story: Evaluation Update, Models, and Measures
11:30 a.m.	Lunch & Farewell Ceremony
12:30 p.m.	Telling the PCORI Engagement Story
1:15 p.m.	Breakout sessions
3:45 p.m.	Report Out
4:15 p.m.	Salesforce Chatter Demonstration
4:45 p.m.	Wrap-up

Welcome, Introductions, and Review Agenda

Jean Slutsky, PA, MSPH

Chief Engagement and
Dissemination Officer

Sue Sheridan, MIM, MBA, DHL

Director of Patient Engagement

Charlotte W. Collins, JD
Chair
Compensation Subcommittee

Darius Tandon, PhD
Chair
Evaluation Subcommittee

New Staff Introductions

Evelyn Whitlock, MD, MPH

Chief Science Officer

Joanna Siegel, MS, ScD

Director of Dissemination and Implementation

Chinenye Anyanwu, PharmD, MPH

Engagement Officer

Sunbo Igho-Osagie, MHSA, CSSGB

Program Associate

William Stewart, Med

Program Associate

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Q&A with Joe Selby

Joe Selby, MD, MPH

Executive Director

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Patient Engagement in Action

Charlotte Collins, JD

Chair

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HEALTH COUNCIL

Advancing Meaningful Patient Engagement

Marc Boutin

Chief Executive Officer

April 11, 2016

Evolution of PDUFA

Initial patient group engagement

Shift to consumer engagement

Patient groups re-engage

1993

PDUFA I:

Backlog reduction

1998

PDUFA II:

FDAMA

Reduce review times

2003

PDUFA III:

BTP

2008

PDUFA IV:

FDAAA

- Unintended consequences
- Process for engagement

2013

PDUFA V:

FDASIA

- Benefit/Risk framework
- Biomarkers/ PROs
- PFDD
- Rare diseases

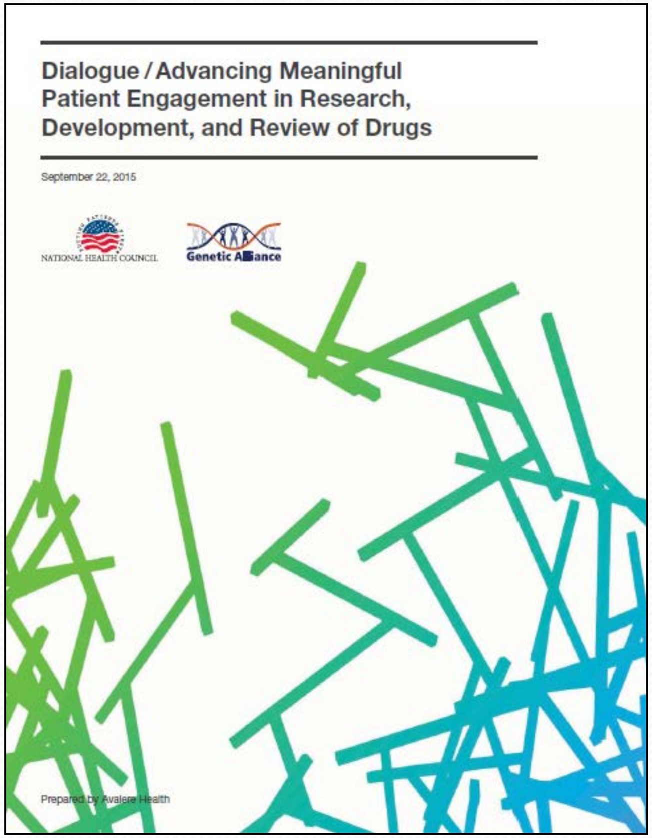
2018

PDUFA VI

Patient engagement throughout the development lifecycle

Dialogue / Advancing Meaningful Patient Engagement in Drug Research, Development & Approval

- Create regulatory guardrails
- Promote a culture shift
- Facilitate open communication



Dialogue / Advancing Meaningful Patient Engagement in Research, Development, and Review of Drugs

September 22, 2015

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Genetic Alliance

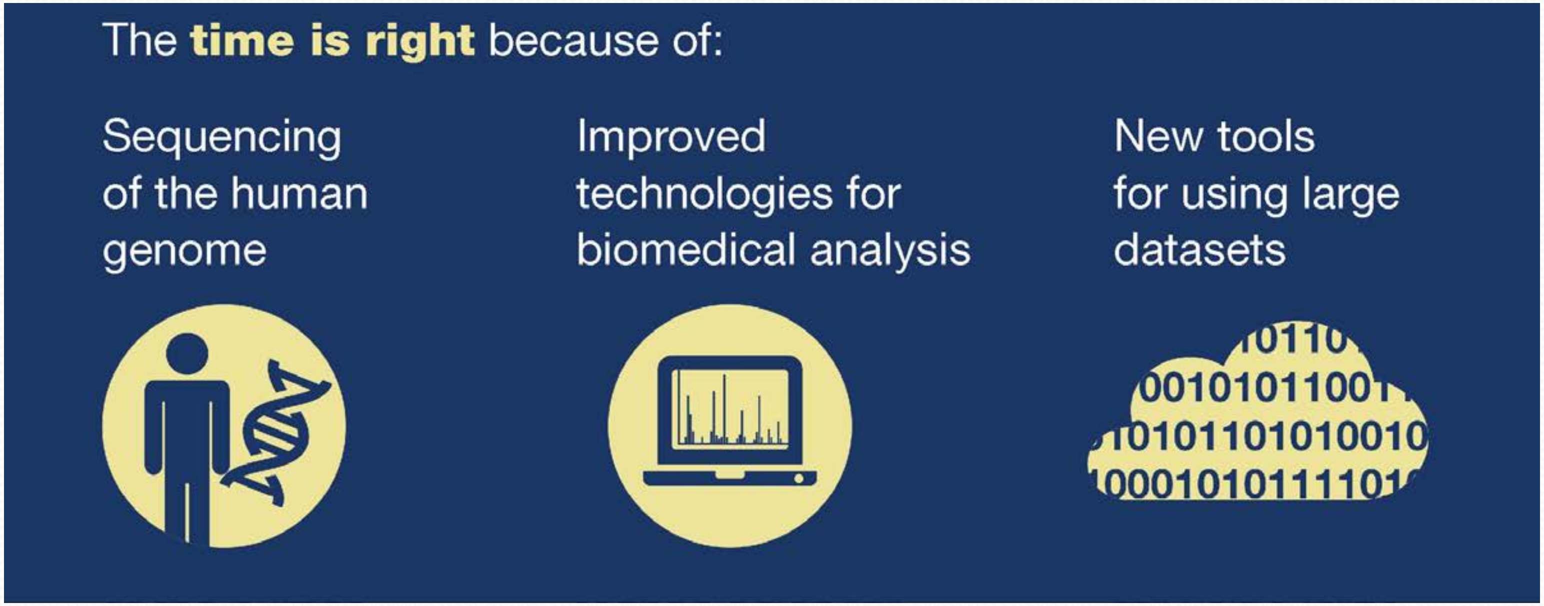
Prepared by Avalere Health

Value

Shifting from Acute to Chronic Care

© Corbis

Shifting from the Average to the Individual



The **time is right** because of:

Sequencing of the human genome

Improved technologies for biomedical analysis

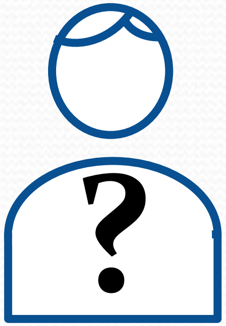
New tools for using large datasets

Source: www.nih.gov/precisionmedicine

Recent Value Models & Frameworks

ASCO®
American Society of Clinical Oncology

ICER
INSTITUTE FOR CLINICAL AND ECONOMIC REVIEW



1884
Memorial Sloan Kettering
Cancer Center

NCCN
National
Comprehensive
Cancer
Network®

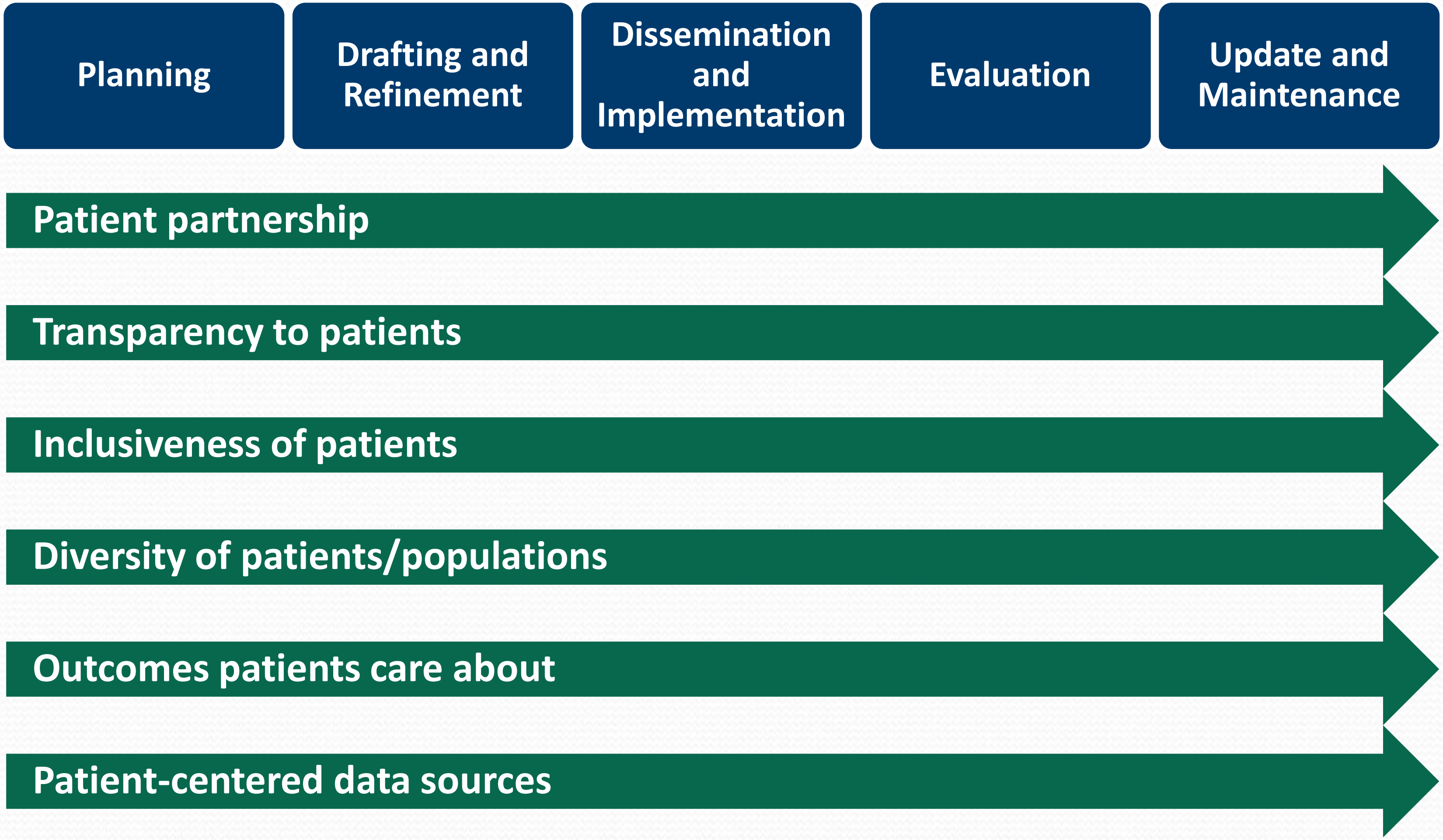
Lack of Obvious Patient Centeredness

No apparent patient engagement throughout the process in

- Defining value
- Developing questions
- Formatting model
- Collecting data
- Dissemination

Costs to patients not consistently assessed and lack of clarity on how it's done

Value-Model Development Process



Planning | Drafting and Refinement | Dissemination and Implementation | Evaluation | Update and Maintenance

- Patient partnership
- Transparency to patients
- Inclusiveness of patients
- Diversity of patients/populations
- Outcomes patients care about
- Patient-centered data sources

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Value Model Rubric: Examples

	Characteristics of Meaningful Patient Engagement in Model Development	Other Characteristics of Patient-Centeredness in Model Development
Patient Partnership	Patients are recognized as partners and are integrated in all aspects of model development phases	Patients are engaged in pilot testing and refinement of the model
Transparency	The process for selection of patient representatives is transparent	The methodology is made transparent to patients in a timely manner
Inclusiveness	The patient community is involved throughout the process	The draft model is vetted with a broad coalition of stakeholders, including patients

Value Model Rubric: Examples

	Characteristics of Meaningful Patient Engagement in Model Development	Other Characteristics of Patient-Centeredness in Model Development
Diversity	Diversity of the patient population is acknowledged and considered	Processes are included for identifying/incorporating new knowledge regarding patient subpopulations and disease trajectory
Outcomes	Outcomes important to patients are identified and incorporated into the mode	Economic inputs are considered in the context of a patient's experience
Data Sources	Existing sources of patient-generated health data are identified and considered	Data beyond randomized controlled trials are considered (e.g., patient preferences)

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Marc Boutin
Chief Executive Officer
National Health Council

mboutin@nhcouncil.org

Break

Patients Count:
The Science of Patient Input

Kim McCleary

Managing Director

FasterCures

Patient Engagement Advisory Panel
Patient-Centered Outcomes Research Institute
April 11, 2016

FasterCures
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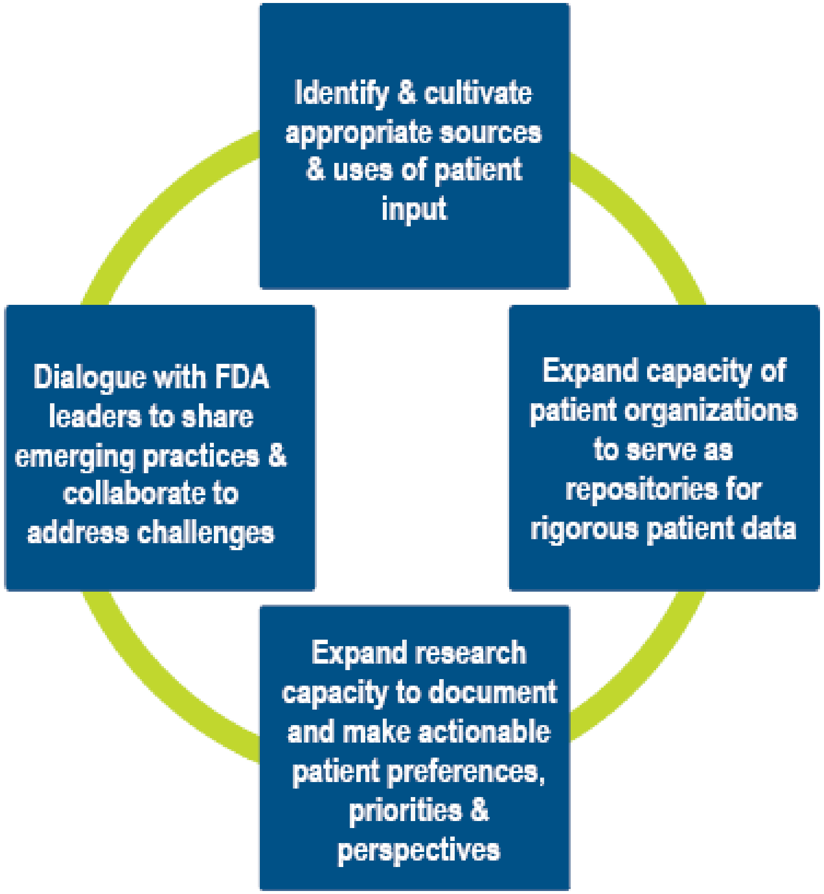
PATIENTS COUNT: THE SCIENCE OF PATIENT INPUT

Expand Thought Leadership

- Share sought-after content expertise through speaking engagements and collaboration in external initiatives
- Foster connections throughout our wide and deep network of diverse stakeholders
- Convene impactful sessions at Partnering for Cures, seasonal workshops, bi-monthly webinars and through the Milken Institute's Global Conference and summits

Enhance Practice



Identify & cultivate appropriate sources & uses of patient input

Dialogue with FDA leaders to share emerging practices & collaborate to address challenges

Expand capacity of patient organizations to serve as repositories for rigorous patient data

Expand research capacity to document and make actionable patient preferences, priorities & perspectives

Improve Policy

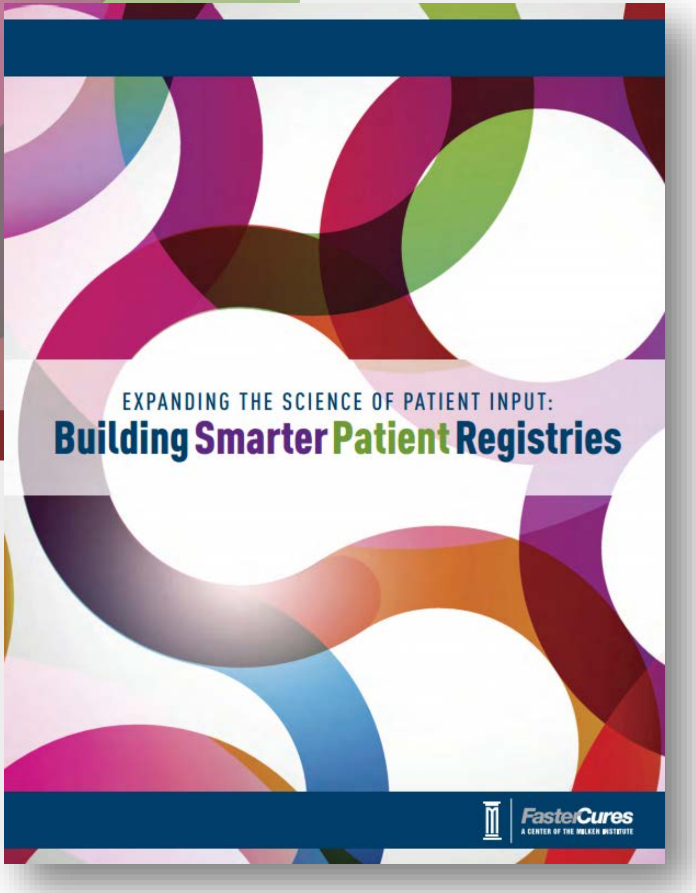
Reinforce principles of patient-centricity that affect access to promising therapies and expand opportunities for patient participation in decision-making, including:

- User-fee negotiations and implementation of user-fee agreements
- Legislative proposals
- Government policy development and implementation

Expanding the Science of Patient Input: Building Smarter Registries

1. Patient registries evolve rapidly.
2. Success = careful planning + active upkeep.
3. Trust is essential, but not enough.
4. Patients expect to be partners.
5. Opportunity will knock. Be prepared.

February 2016



EXPANDING THE SCIENCE OF PATIENT INPUT:
Building Smarter Patient Registries

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PATIENTS COUNT: THE SCIENCE OF PATIENT INPUT

Science Translational Medicine AAAS

FOCUS

POLICY

From passengers to co-pilots: Patient roles expand

Margaret Anderson* and K. Kimberly McCleary*

The premier position of medical research on the U.S. national policy agenda offers an unprecedented opportunity to advance the science of patient input and marks a turning point in the evolution of patient engagement.

For most of history, patients have been the passive recipients of medical care with little or no role in research. Even as research subjects, patients were not required to give informed consent prior to adoption of the Nuremburg Code in 1947. Since then, patient participation has expanded dramatically, and today, opportunities abound to serve as active partners in defining and prioritizing research questions and solutions. As digital strategist Leonard Kish declared in 2012, "If patient engagement were a drug, it would be the blockbuster drug of the century and malpractice not to use it" (*1*).

Patient engagement offers the promise of advancing more personal and efficacious medical products faster than the typical ~15-year discovery-to-market timeline (*2*). Here, we explore the early foundations of patient engagement (table S1), where it occurs in the drug-development pipeline, the power of recent policy initiatives, and prospects for success in improving health outcomes.

FROM SIDELINES TO CENTER COURT

Early in the last century, patients began to mobilize to accelerate research for particular conditions. The March of Dimes, founded by President Franklin D. Roosevelt in 1938 to expand polio research, is one of the first examples of philanthropy directed at finding treatments and cures. Research supported by individuals through the March of Dimes led to development of the "iron lung" and a successful vaccine. Until recently, this case was an outlier, considering that until the 1973 Patient Bill of Rights was adopted by the American Hospital Association, patients did not necessarily expect to be told their diagnosis, much less have a voice in determining their care plan.

Addressing a patient's part in advancing biomedicine.

CREDITS: LIBRARY OF CONGRESS, FROM GOVERNMENT, MURAL BY E. VEDDER; PHOTOGRAPHED BY C. HIGHSMITH; QUOTE GETTYSBURG ADDRESS (1863)

Even in recent years, patients didn't always express their own preferences and expectations for care, deferring to choices the doctor deemed best.

The HIV/AIDS movement catapulted patient needs to the forefront of research and created the force for change that dramatically altered regulatory approval processes at the U.S. Food and Drug Administration (FDA), funding formulas and emphasis at the U.S. National Institutes of Health (NIH), and the path forward for disease organizations. People affected by HIV rallied together and created a movement that demanded change and got results (*3*): from the creation of Gay Men's Health Crisis in New York in 1982 and the AIDS Coalition to Unleash Power in 1987, to the National Institute of Allergy and Infectious Diseases' (NIAID's) formation of the largest HIV clinical trials network in the world, to protests at both NIH and FDA, to passage of the Ryan White Comprehensive AIDS Resources Emergency Act in 1990.

The HIV/AIDS model continues to provide a roadmap followed by other patient communities, demonstrating that it is not enough to question the status quo; you have to do the hard work of presenting well-founded alternatives. As Anthony Fauci, director of NIAID, noted at a *FasterCures* event in 2011, "If you really want to shake cages you have to be persistent. This is very different than coming to a meeting once a year. We knew the HIV/AIDS activists weren't going away."

Today, the role of patients as partners permeates the R&D landscape, extending far beyond the traditional model of funding basic science through donations. Spurred on by the increase of entrepreneurial philanthropy and the proliferation of technology that connects and empowers patient communities, patient influence on decision-making is increasing. In particular, the venture philanthropy drug-development model pioneered by the Cystic Fibrosis Foundation—which led to the codevelopment, with Vertex Pharmaceuticals, of Kalydeco, the first disease-modifying treatment aimed at the genetic cause of cystic fibrosis—is gaining steam and altering the landscape of disease research and cross-sector collaboration.

FasterCures, Washington, DC 20005, USA.
*Corresponding author. E-mail: manderson@fastercures.org (M.A.); kmccleary@fastercures.org (K.K.M.)

June 10, 2015

At press – for publication in April 2016:

"On the pathway to a science of patient input: Where are we now and what lies ahead?"

Margaret A. Anderson & K. Kimberly McCleary

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2016 Landscape Assessment:

More than 70 collaborative initiatives to advance patient-centricity

- Frameworks & Models
- Methods & Toolkits
- Sources of Patient Data
- Regulatory & Legislative Activity
- Training Programs
- Measurement & Metrics

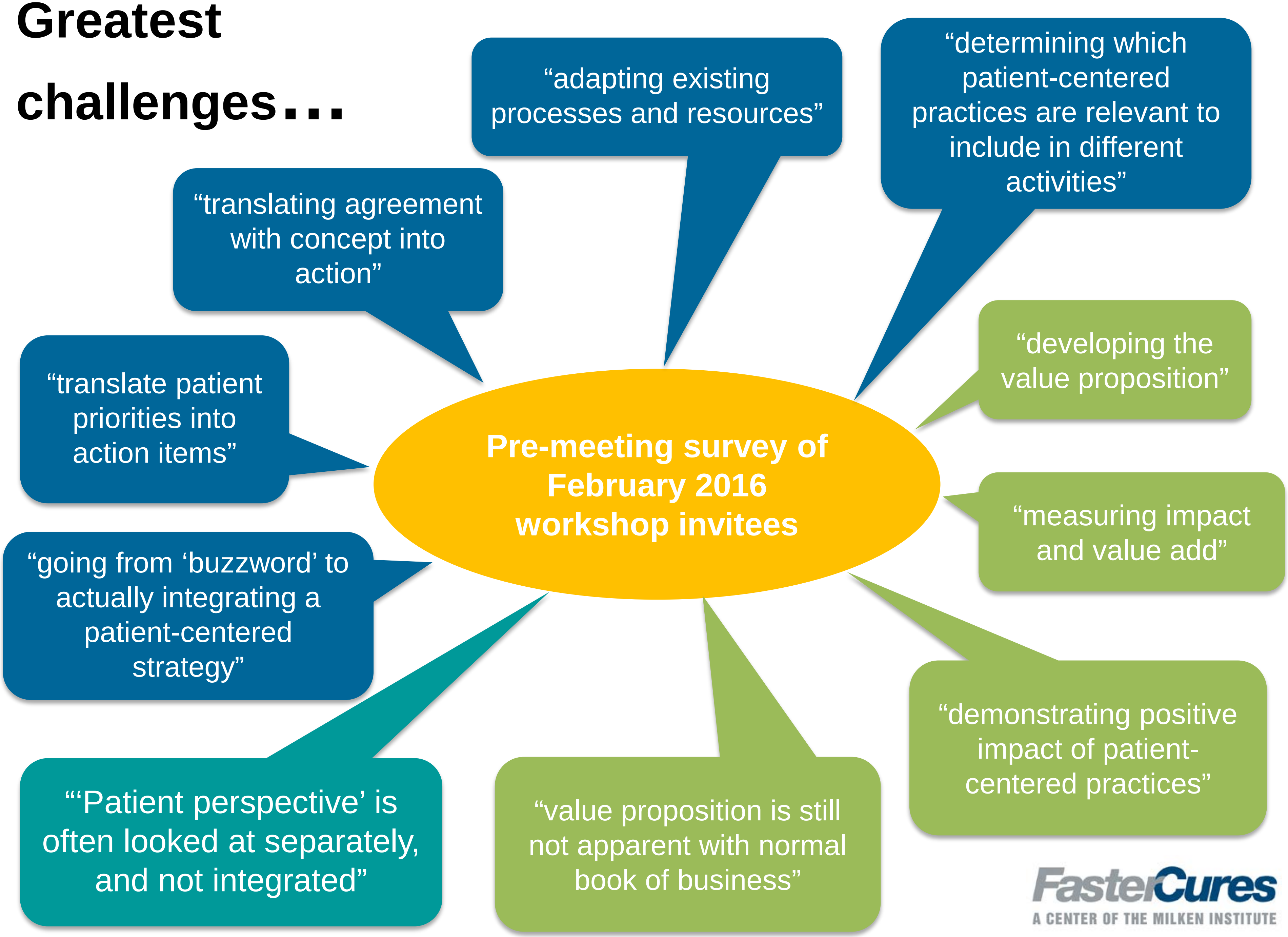
On-line version of our upcoming April 2016 *Science Translational Medicine* article will include a directory of these 70+ initiatives (with live links) that we will update regularly

Operational Tools to Support Expanded Patient Engagement

February 16-17, 2016

With thanks to Pfizer for its support

Greatest challenges…



Pre-meeting survey of February 2016 workshop invitees

“adapting existing processes and resources”

“determining which patient-centered practices are relevant to include in different activities”

“translating agreement with concept into action”

“translate patient priorities into action items”

“going from ‘buzzword’ to actually integrating a patient-centered strategy”

“developing the value proposition”

“measuring impact and value add”

“demonstrating positive impact of patient-centered practices”

“value proposition is still not apparent with normal book of business”

“‘Patient perspective’ is often looked at separately, and not integrated”

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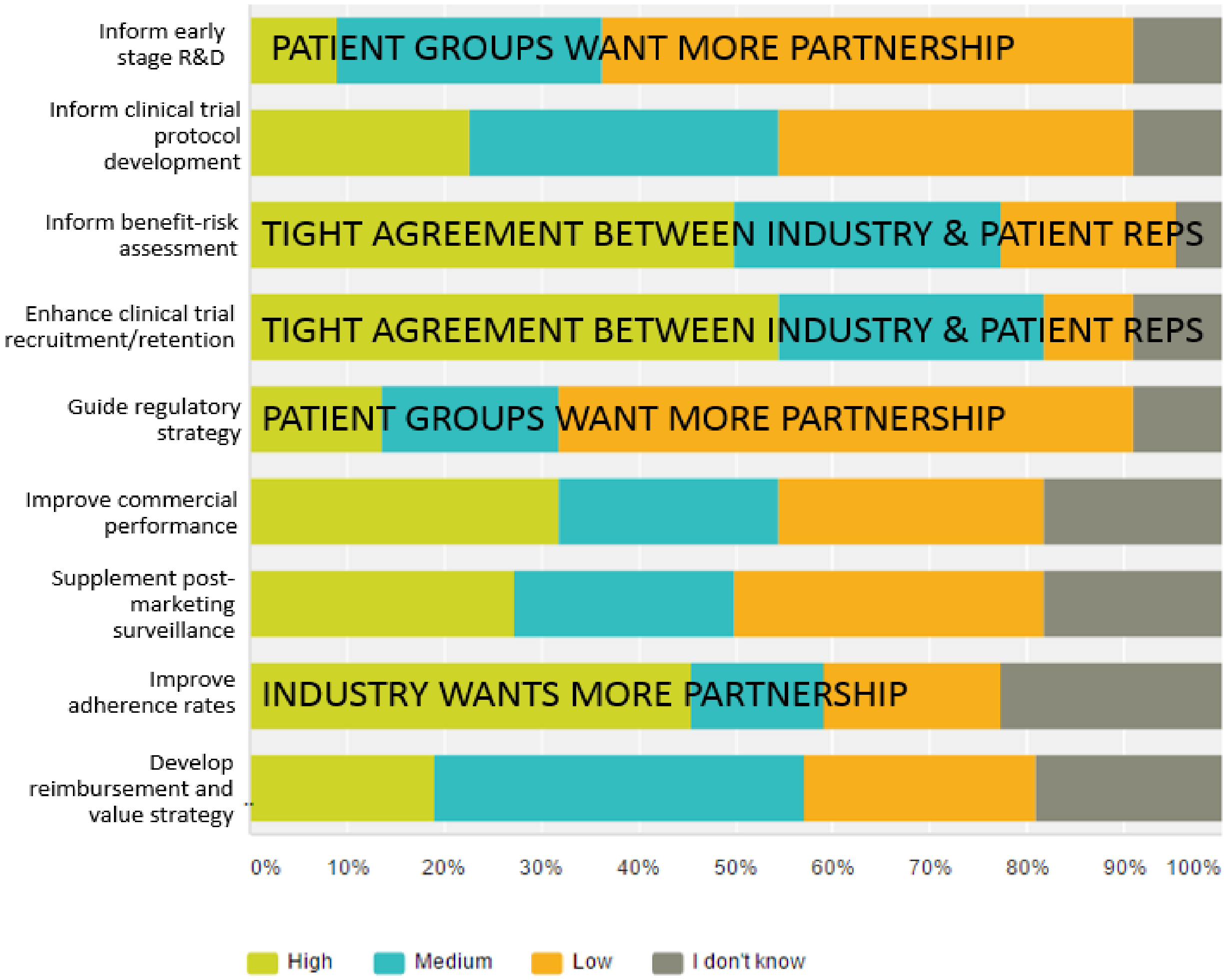
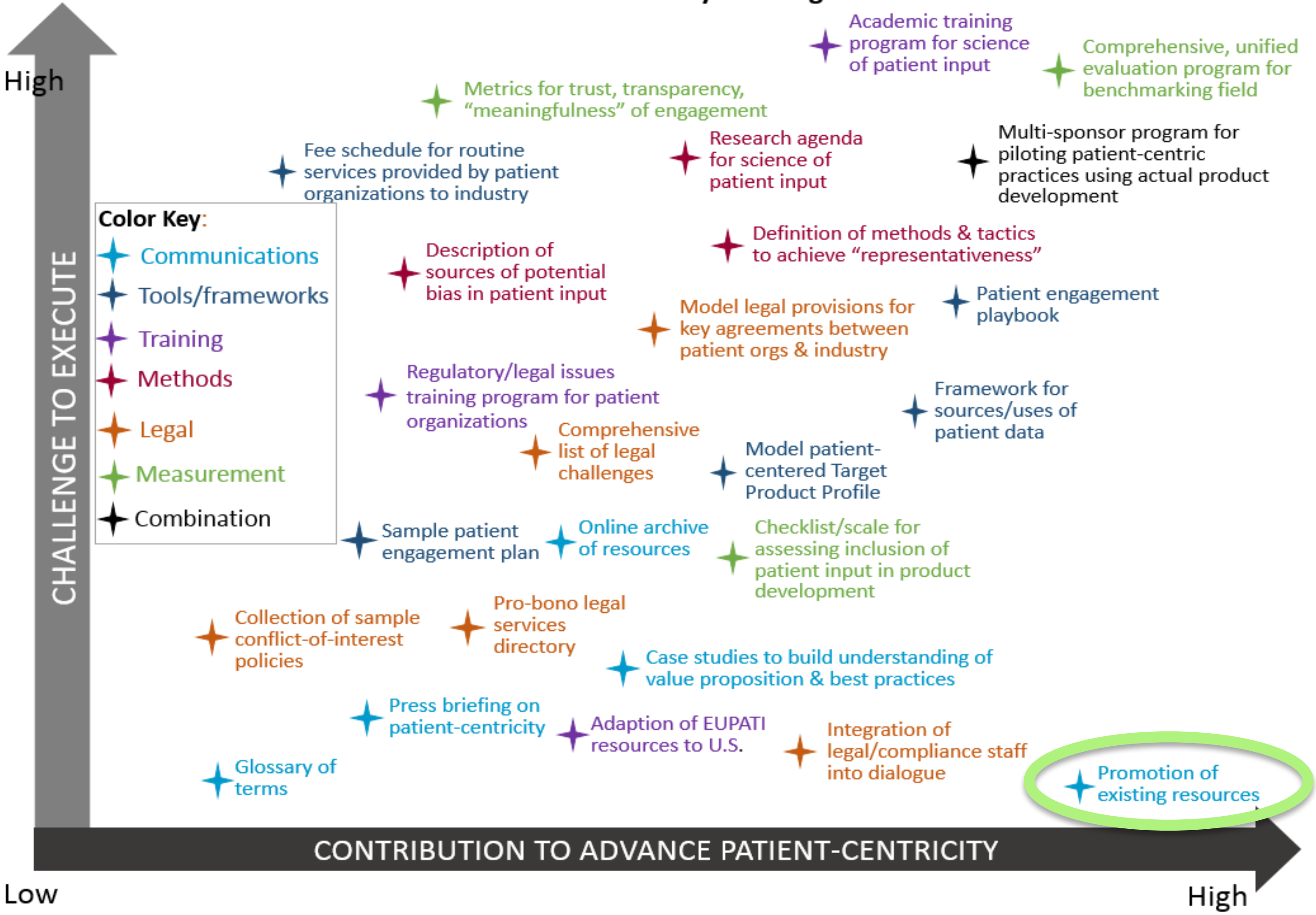
Perceived interest in industry for integrating patient perspectives at various stages of medical product development

Bars represent all participants' responses; areas of tight agreement and discord noted with text



Workshop report to be published in late April 2016: **“Expanding the Science of Patient Input: Pain Points and Potential”**

Relative classification of collaborative initiatives by challenge in execution and contribution to field



High

CHALLENGE TO EXECUTE

CONTRIBUTION TO ADVANCE PATIENT-CENTRICITY

Low

High

Color Key:
- Communications
- Tools/frameworks
- Training
- Methods
- Legal
- Measurement
- Combination

Academic training program for science of patient input

Comprehensive, unified evaluation program for benchmarking field

Metrics for trust, transparency, “meaningfulness” of engagement

Fee schedule for routine services provided by patient organizations to industry

Research agenda for science of patient input

Multi-sponsor program for piloting patient-centric practices using actual product development

Definition of methods & tactics to achieve “representativeness”

Description of sources of potential bias in patient input

Patient engagement playbook

Model legal provisions for key agreements between patient orgs & industry

Regulatory/legal issues training program for patient organizations

Framework for sources/uses of patient data

Comprehensive list of legal challenges

Model patient-centered Target Product Profile

Sample patient engagement plan

Online archive of resources

Checklist/scale for assessing inclusion of patient input in product development

Collection of sample conflict-of-interest policies

Pro-bono legal services directory

Case studies to build understanding of value proposition & best practices

Press briefing on patient-centricity

Adaption of EUPATI resources to U.S.

Integration of legal/compliance staff into dialogue

Promotion of existing resources

Glossary of terms

Health IT Joint Committee Collaboration

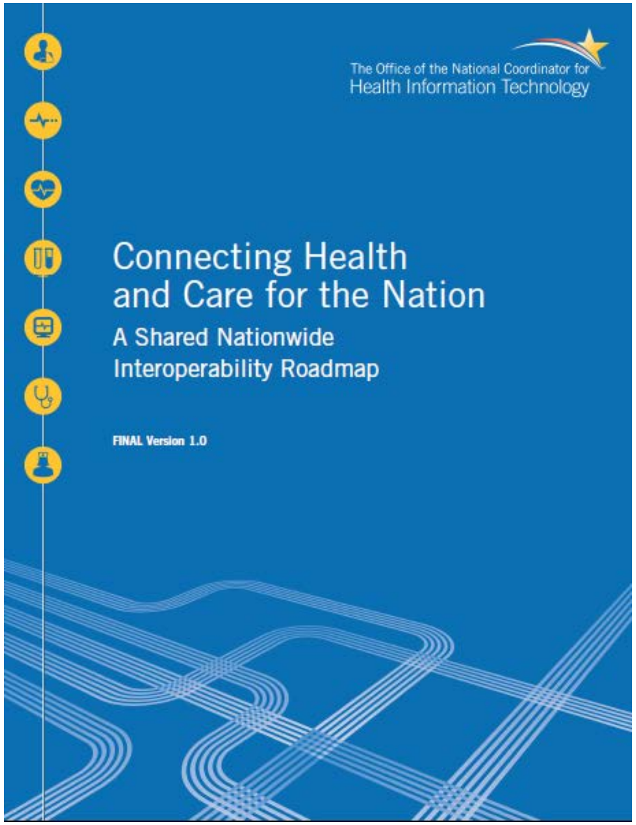
A Joint Policy and Standards Public Advisory Body on Health Information Technology
to the National Coordinator for Health IT

Interoperability Experience Task Force

Jitin Asnaani, co-chair
Anjum Khurshid, co-chair

Nationwide Interoperability Roadmap

- Strong flexible health IT ecosystem that supports transparency and decision making, reduces redundancy, informs payment reform and helps transform care . . .
- An interoperable health IT ecosystem makes the right data available to the right people at the right time across products and organizations . . .



The Office of the National Coordinator for
Health Information Technology

Connecting Health
and Care for the Nation

A Shared Nationwide
Interoperability Roadmap

FINAL Version 1.0

Federal Advisory Committees

- The American Recovery and Reinvestment Act of 2009 (ARRA) provided for the creation of an HIT Policy Committee and an HIT Standards Committee under the auspices of the Federal Advisory Committee Act (FACA)
- FACA committees make recommendations to the National Coordinator for Health IT on
  - policy framework for the development and adoption of a nationwide health information infrastructure
  - standards, implementation specifications, and certification criteria

Joint HITPC/HITSC Task Force's Charge

Provide recommendations on the most impactful policy, technical, and public-private approaches that could be implemented to improve the interoperability experience for providers and patients.

- Assume that the stakeholder has access to a system(s) that can interoperate with at least one other system from outside
- Identify the top 3 to 5 most important needs for these stakeholders
- Narrow the scope of work to the most impactful actions
- Make specific/actionable recommendations for ONC, in collaboration with others (e.g., standards bodies, commercial parties and other Federal entities)

Patient Engagement in HIT

- Individual empowerment and patient engagement are key principles identified in the *Nationwide Interoperability Roadmap* and in the *Health IT Strategic Plan*

Goal 1
Advance Person-Centered and Self-Managed Health

Goal 2
Transform Health Care Delivery and Community Health

Goal 3
Foster Research, Scientific Knowledge, and Innovation

Goal 4
Enhance Nation's Health IT Infrastructure

Interoperability Experience Task Force

Member	Last Name	Organization
Co-Chairs		
Jitin	Asnaani	CommonWell Health Alliance
Anjum	Khurshid	Louisiana Public Health Alliance
Members		
Kelly	Aldrich	Center for Medical Interoperability
John	Blair	MedAllies
Janet	Campbell	Epic
George	Cole	Allscripts
Ty	Faulkner	Rural Health Information Technology
Larry	Garber	Reliant Medical Group, Atrius Health
Shaun	Grannis	Regenstrief
Jane	Perlmutter	Patient Advocate
Phil	Posner	Patient Advocate
Cris	Ross	Mayo Clinic
Larry	Wolf	Strategic Health Network
Federal Ex-Officio		
Jorge	Ferrer	Veterans Health Administration
ONC Staff		
Stacy	Perchem	ONC Staff Lead

Framework for Capturing Insights

Identify the top 3 to 5 most important interoperability needs for these stakeholders:

1. **Needs not being achieved**
2. **Needs being partially achieved, but still significant obstacles**
   e.g., clinical experience, operational experience/TCO, or ability to scale
3. **Needs fully addressed**
   (minor gaps may exist)

Illustrative Needs Matrix

	Needs	**Need being fulfilled?** (Options 1-3 above)	**Impact** (Low / Medium / High) + Reason
1	<Need #1>	Not being achieved	High – patient safety issue
2	<Need #2>	Being partially achieved	Medium – impedes workflow
3	<Need #3>	Fully addressed	Low – enhances UX in specific situations

DRAFT Work plan

Meeting Dates	Task
Tue, Mar 8, 2016 - 3:00pm ET	Task Force Kickoff Needs identified by Task Force
Wed, Mar 23, 2016 - 1:00pm ET	Planning for virtual hearing panel discussions
Wed, Apr 6, 2016 - 1:00pm ET	Virtual Hearing #1
Wed, Apr 20, 2016 - 1:00pm ET	Virtual Hearing #2
Fri, May 6, 2016 - 10:30am ET	Summarize & discuss needs & solutions identified by Panelists
Wed, May 11, 2016 - 10:30am ET	Draft recommendations for prioritized needs
Wed, Jun 1, 2016 - 1:00pm ET	Recommendations refinement
June 7/8 HITPC/HITSC Meetings	***Draft Recommendations presented to HITPC/HITSC***
TBD - needs to scheduled	Revise and edit recommendations
July 12/13 HITSC/HITPC Meetings	***Recommendations presented to HITSC/HITPC***
July 29	***Transmittal letter sent to the National Coordinator***

Q&A

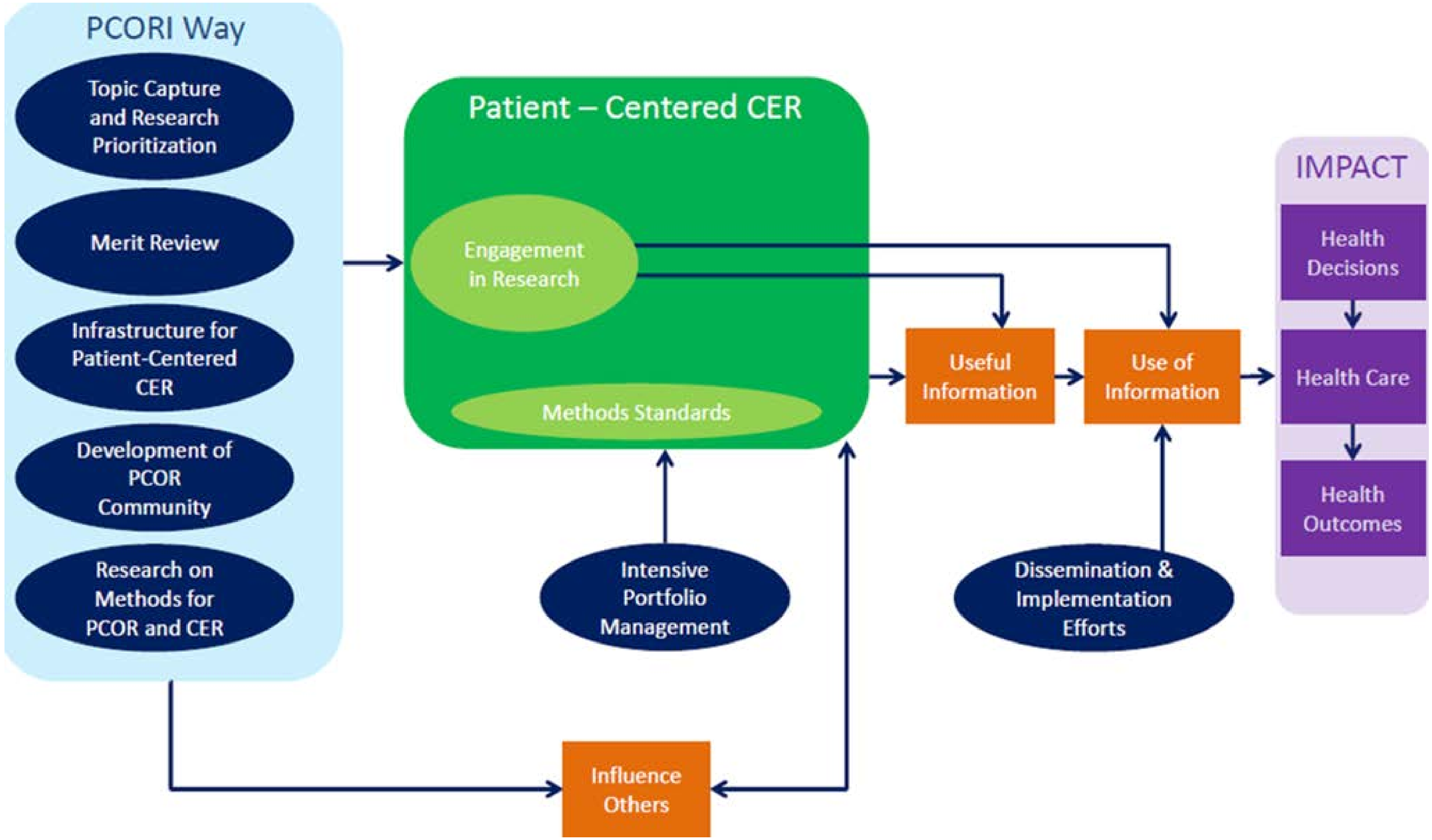
Evaluating PCORI’s Impact: The Role of Research Engagement

Laura Forsythe, PhD, MPH

Associate Director, Evaluation and Analysis

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Evaluating the Impact of PCORI: Where does Engagement fit?



PCORI Way

Topic Capture and Research Prioritization

Merit Review

Infrastructure for Patient-Centered CER

Development of PCOR Community

Research on Methods for PCOR and CER

Patient – Centered CER

Engagement in Research

Methods Standards

Useful Information

Use of Information

IMPACT

Health Decisions

Health Care

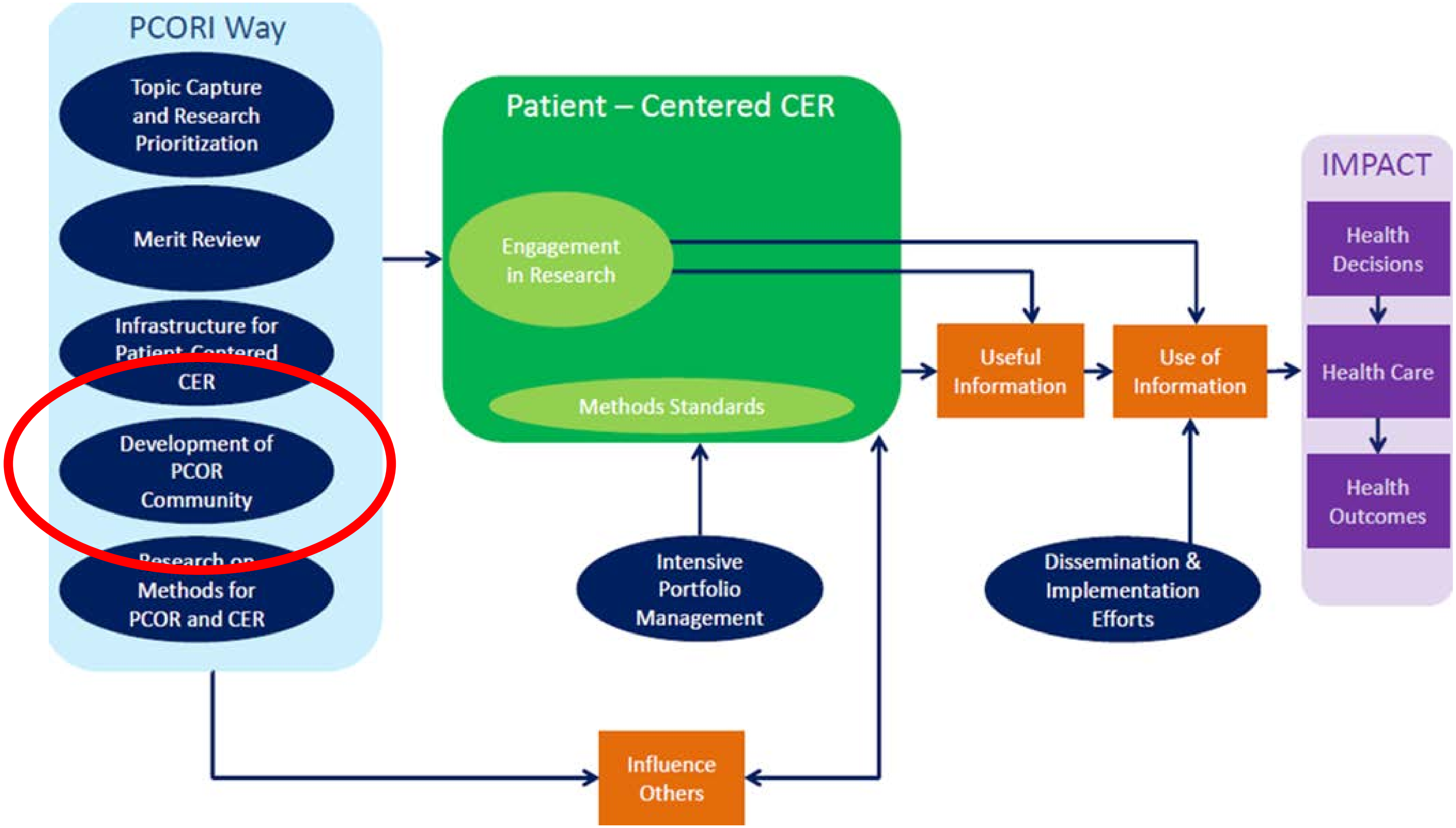
Health Outcomes

Intensive Portfolio Management

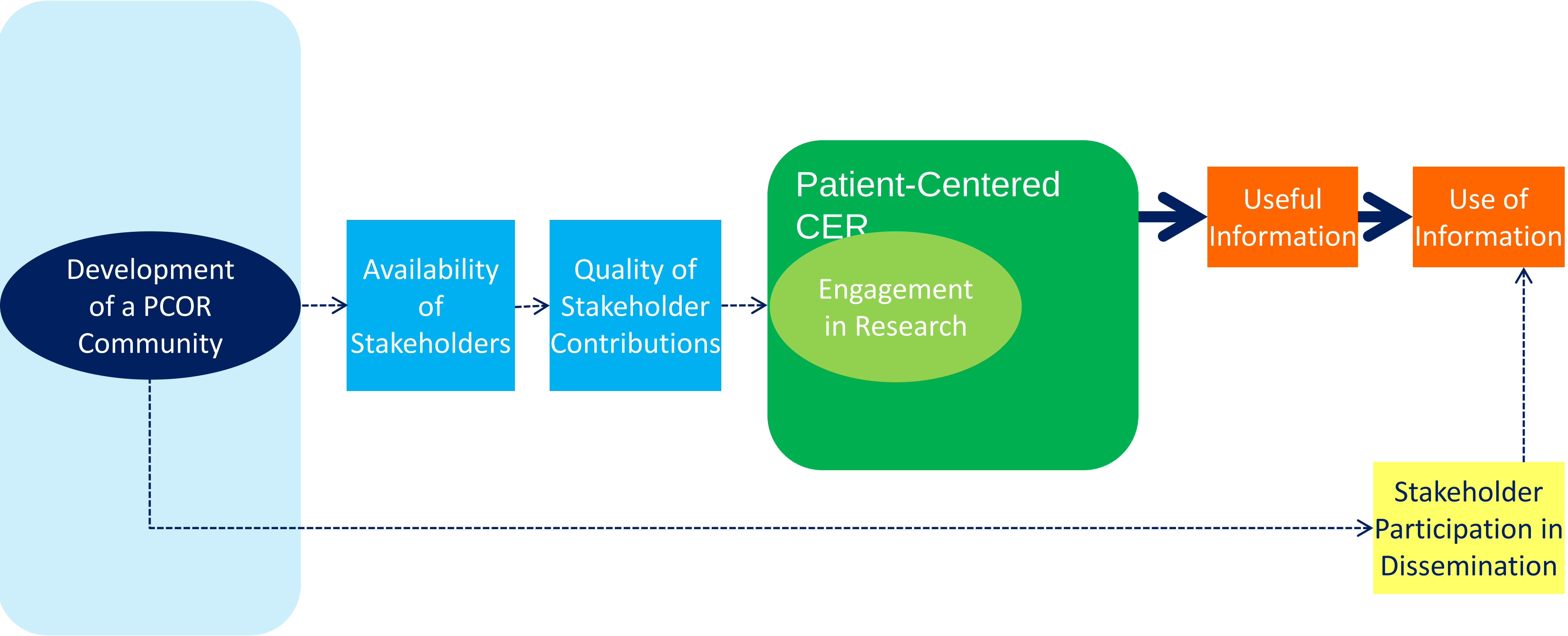
Dissemination & Implementation Efforts

Influence Others

Evaluating the Impact of PCORI: Where does Engagement fit?



Development of a PCOR Community



Development of a PCOR Community

Availability of Stakeholders

Quality of Stakeholder Contributions

Patient-Centered CER

Engagement in Research

Useful Information

Use of Information

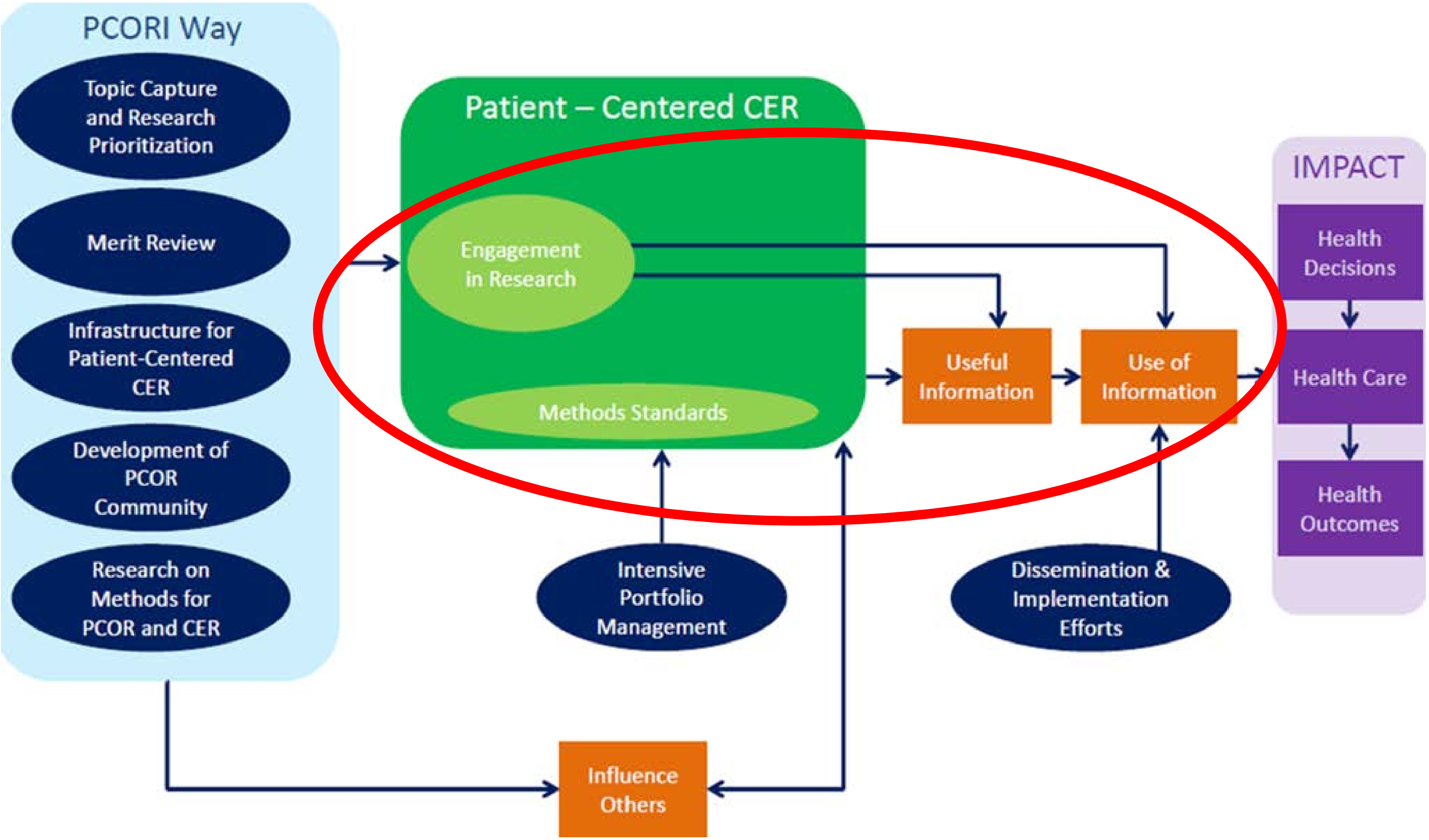
Stakeholder Participation in Dissemination

Primary Evaluation Questions

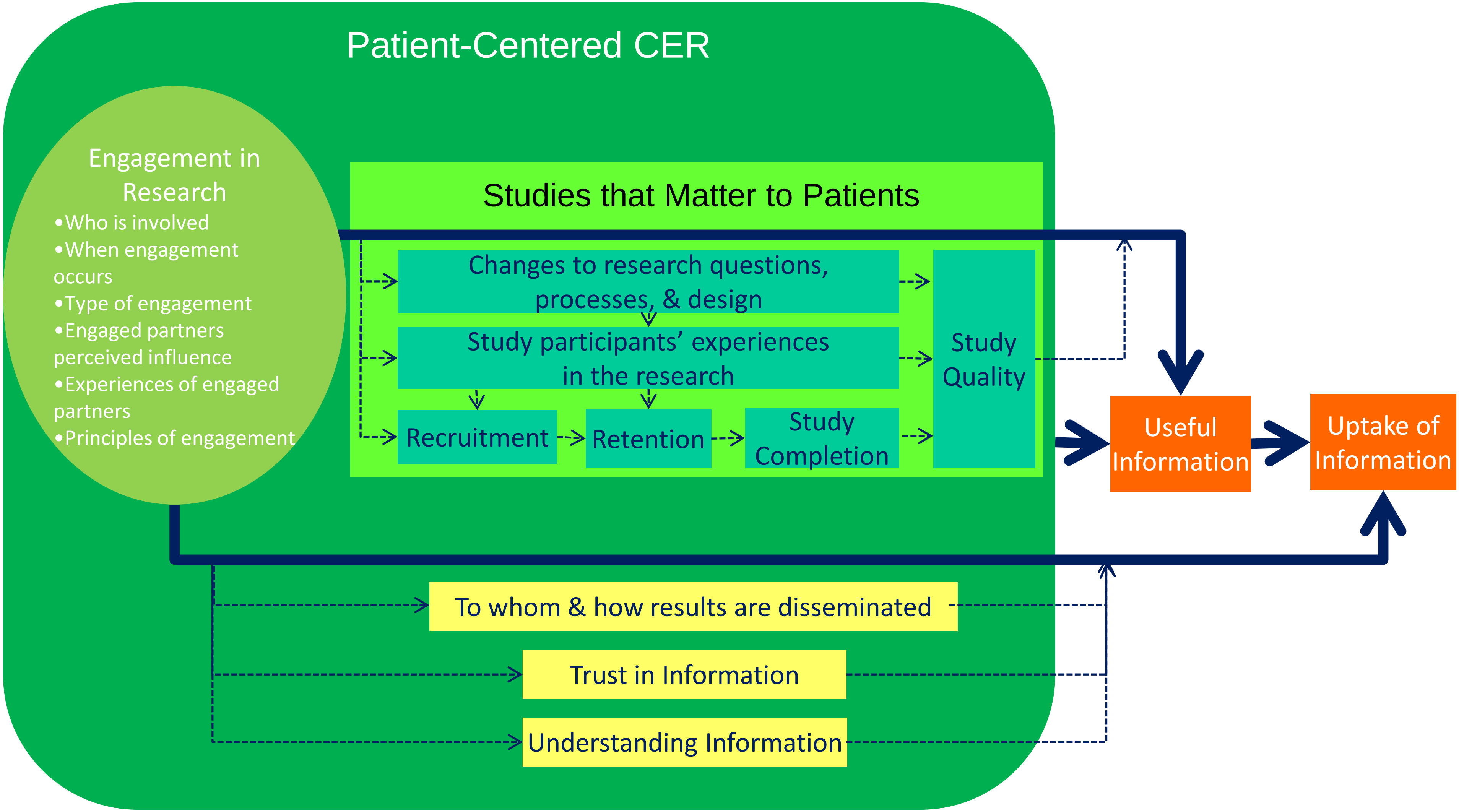
What is the effect of PCORI’s unique approach to **developing a PCOR community**?

- Number of trained merit reviewers, ambassadors, and advisory panelists
- Number of people attending PCORI events and accessing PCORI training materials
- Number of applications that come from the Pipeline to Proposal Awards
- Public perceptions of, and willingness to, engage in Patient-Centered CER projects
- Researcher perceptions of difficulty finding partners
- Stakeholder perceptions of difficulty finding opportunities for partnering in research
- Researcher and stakeholder interest in Patient-Centered CER and awareness of methods for Patient-Centered CER
- Input from patients and stakeholder partners perceived as influential, valuable

Evaluating the Impact of PCORI: Where does Engagement in Research fit?



Engagement in Research



Patient-Centered CER

Engagement in Research

- Who is involved
- When engagement occurs
- Type of engagement
- Engaged partners perceived influence
- Experiences of engaged partners
- Principles of engagement

Studies that Matter to Patients

Changes to research questions, processes, & design

Study participants' experiences in the research

Recruitment

Retention

Study Completion

Study Quality

Useful Information

Uptake of Information

To whom & how results are disseminated

Trust in Information

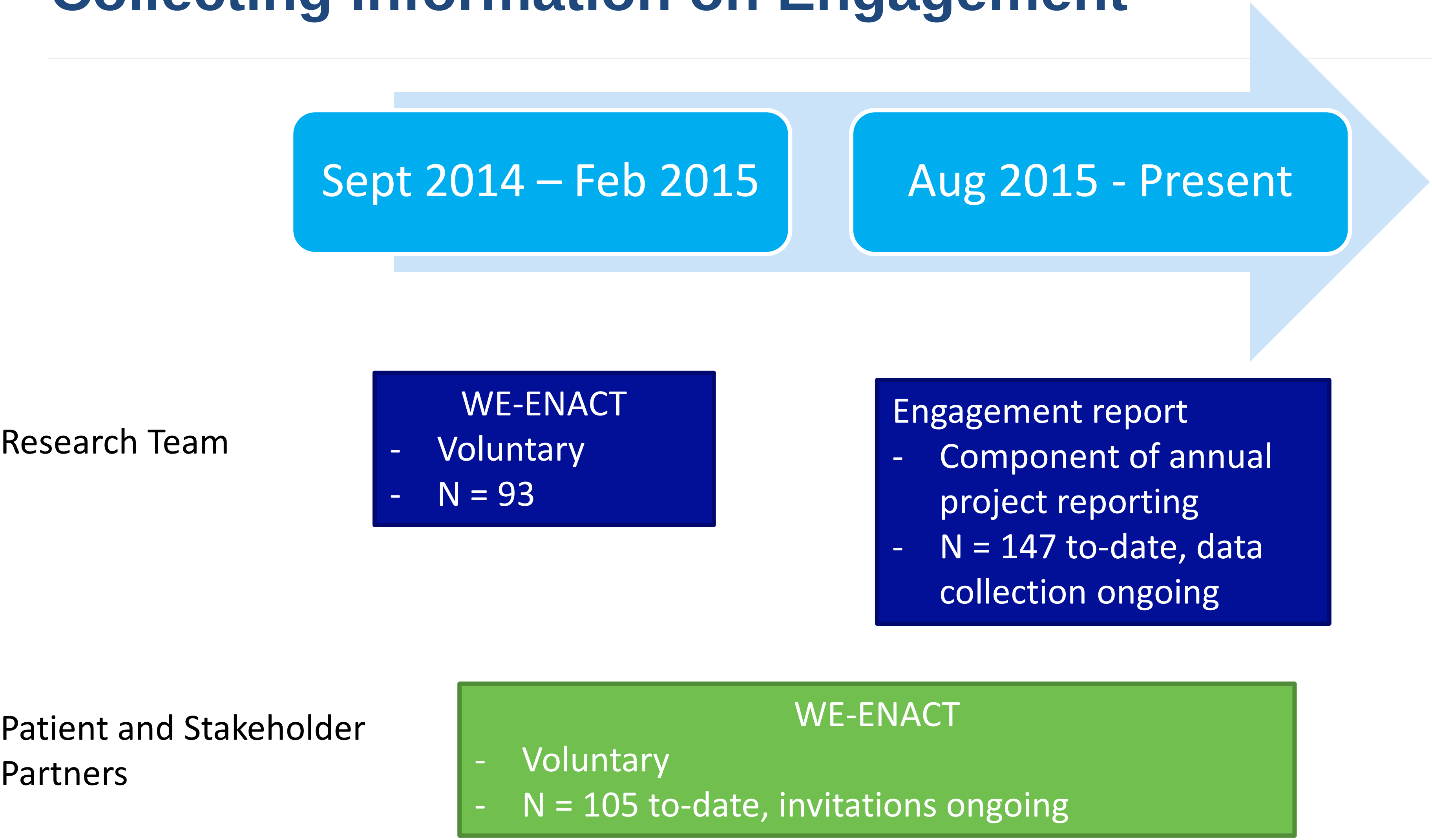
Understanding Information

Primary Evaluation Questions

What is the effect of **Engagement in Research** on…?

- Relevance of research questions for end-users
- Changes to study design resulting from engagement
- Recruitment and retention rates
- Proportion of studies that complete data collection
- Time to study completion
- To whom and how research is disseminated
- Time to dissemination
- Perceptions of influence among research partners
- Trust in results
- Understanding of findings
- Study quality

Collecting Information on Engagement



Sept 2014 – Feb 2015

Aug 2015 - Present

Research Team

WE-ENACT
- Voluntary
- N = 93

Engagement report
- Component of annual project reporting
- N = 147 to-date, data collection ongoing

Patient and Stakeholder Partners

WE-ENACT
- Voluntary
- N = 105 to-date, invitations ongoing

Information on Engagement: Domains

- Who is engaged
- When they are engaged
- How they are engaged
- Level of engagement (information, consultation, collaboration, Patient/stakeholder direction)
- Influence of patients and other stakeholders
- Impact on the project
- Challenges and how they were overcome
- Activities and progress relative to engagement plan

Example Findings: Changes to Study Design

- Changes to study design to make it more responsive to patient needs, feasible in clinical setting
- Some researchers reported minimal impact on study design

"High impact - changed design, outcomes, flow of study." - *Researcher*

"Contributed to the approach taken and to creating conditions that would allow maximum participation on the part of both patients and providers." – *Patient/Stakeholder partner*

"This led us to modify our original 2-group research design and include a 3rd group; community based group exercise." - *Researcher*

"The timeline of study assessments was modified in response to stakeholder feedback." - *Researcher*

Example Findings: Recruitment & Retention

- Recruitment procedures more responsive to patient needs
- Changes to recruitment messages
- More potential participants aware of the study
- Improvements in recruiting and retaining difficult-to-reach populations

"Outreach materials, recruitment procedures were modified significantly." - *Researcher*

"Since discussing our challenges with recruiting and retaining study participants, we have had only one participant decline to participate." - *Researcher*

Other sources of information on Engagement

- Qualitative review of additional information in Interim Progress Reports
- In-depth interviews
- Awardee publications as case studies

Reports from the Field

Engaging stakeholders to design a comparative effectiveness trial in children with uncontrolled asthma

Journal of Comparative Effectiveness Research

Kim Erwin[*,1], Molly A Martin[2], Tara Flippin[1], Sarah Norell[1], Ariana Shadlyn[1], Jie Yang[1], Paula Falco[1], Jaime Rivera[1], Stacy Ignoffo[3], Rajesh Kumar[4], Helen Margellos-Anast[5], Michael McDermott[6], Kate McMahon[7], Giselle Mosnaim[8], Sharmilee M Nyenhuis[2], Valerie G Press[9], Jessica E Ramsay[5], Kenneth Soyemi[10], Trevonne M Thompson[2] & Jerry A Krishnan[2,11]

Contents lists available at ScienceDirect

Contemporary Clinical Trials

journal homepage: www.elsevier.com/locate/conclintrial

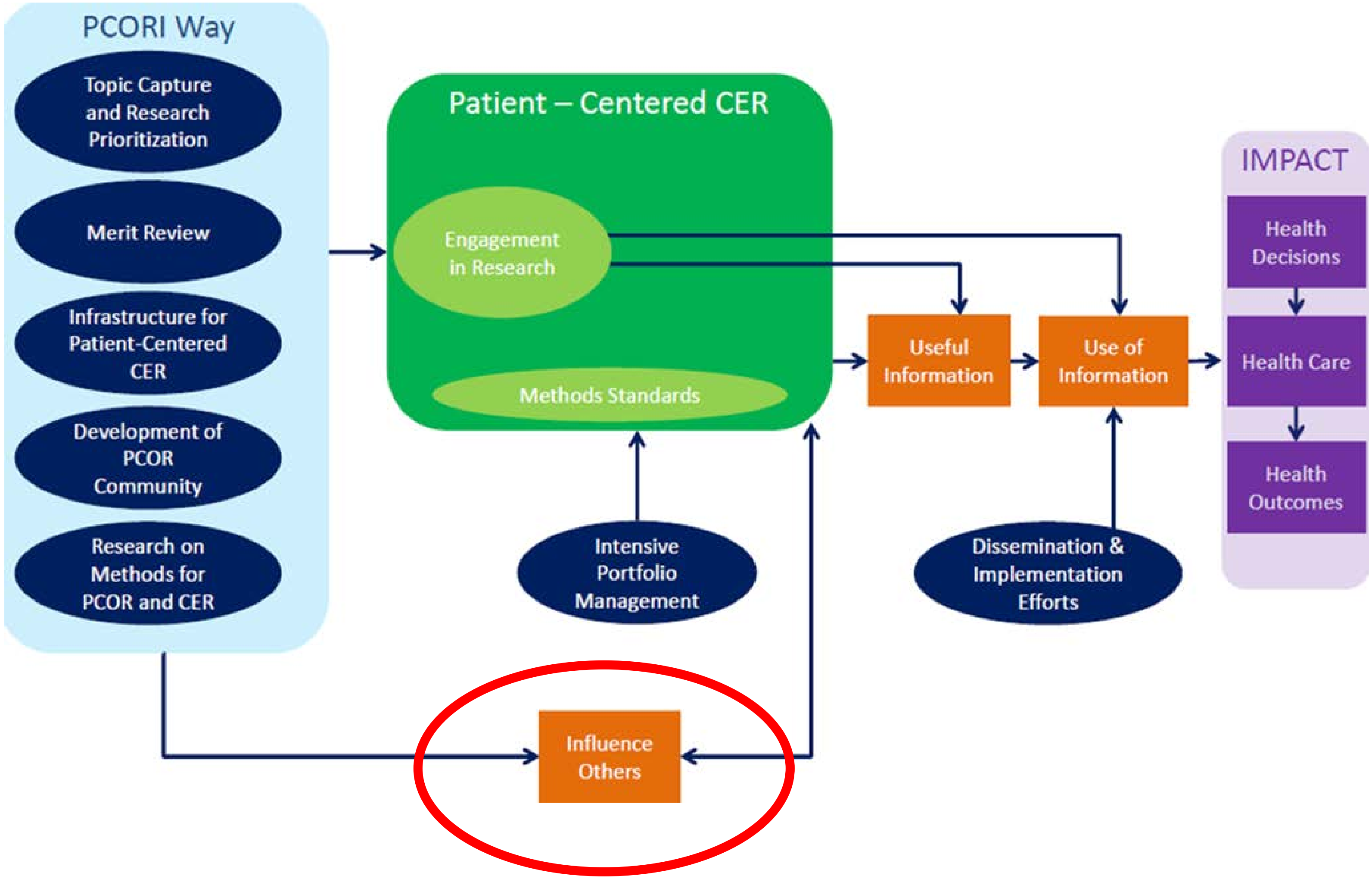
ELSEVIER

Using patient engagement in the design and rationale of a trial for women with depression in obstetrics and gynecology practices☆

CrossMark

Ellen Poleshuck[a,b,*], Marsha Wittink[a], Hugh Crean[c], Tara Gellasch[d], Mardy Sandler[e], Elaine Bell[a], Iwona Juskiewicz[a], Catherine Cerulli[a]

Evaluating the Impact of PCORI: Where does Engagement in Research fit?



Primary Evaluation Questions

How does PCORI's work **influence others**?

- Use of CER and PCOR terminology
- Endorsement, promotion, and dissemination of PCORI work
- Bibliometric indicators of PCORI evaluation work, guidance on patient-centered CER, and methods projects
- Other funders use of the following criteria in proposal review: patient-centeredness, engagement, potential speed of uptake of findings in clinical practice
- Use of non-scientists in proposal review
- Use of training or curricula developed or funded by PCORI
- Number and nature of projects co-funded by PCORI and others
- Number and proportion of studies conducted in PCORnet by investigators external to PCORnet
- Use of PCORI methodology standards on patient-centeredness in non-PCORI research
- Amount and proportion of total PCOR funding that comes from funders other than PCORI

Influence Example #1

University of Texas Health Science Center at San Antonio

PCORI is credited with motivating:*

- **Workshops** on PCORI
    - A listserv, working group focused on PCORI applications (~130 investigators), and day-long in-service on grant writing
- Clinical Investigator Kick-start (CLIK) **awards**
    - $50K, one year
    - Identified as a priority area the funding of meaningful engagement with partners, to increase knowledge about and skills in research engagement
- **New policies** to permit hiring patient or stakeholder partners as experts on university pay roll
- **Patient-centered approaches** to applications for research to other funders

"I plan to use the model of doing research in partnership with patients who have the illness and community health centers for everything going forward." *Dawn Velligan*

**Jennifer Potter, PhD, MPH, Assistant Dean for Research and Student Programs*

Influence Example #2

University of Pittsburgh Comparative Effectiveness Research Center (CERC)

PCORI is credited with motivating:

- Establishment of a HIPAA compliant **data center:**
  - 20 projects currently using it, $13 million across all projects (PCORI and other funders)
- Development of **training and educational opportunities**:
  - Graduate courses & training grants (AHRQ-funded) based on the PCORI Methodology Standards
  - 54 training workshops since 2011 on PC-CER funding opportunities and review criteria, PC-CER methodology, and stakeholder engagement
  - Mock reviews for PCORI applications (assess engagement, adherence to standards)
- Emphasis on **stakeholder engagement:**
  - *"These are new concepts for some of our researchers – PCORI is making them think about the stakeholders and how they can qualify to be a PCORI project" – Monica Costlow, CERC Project Director*

"PCORI is central to the CERC and has greatly influenced work across the University"

Sally Morton, Director of CERC and PCORI Methodology Committee Member

Lunch
&
Farewell Ceremony

Charlotte Collins

Kristin Carman

Perry Cohen

Bruce Hanson

Amy Kratchman

Kim McCleary

Julie Moretz

Sally Okun

Telling the PCORI Engagement Story: Engagement Overview

Sue Sheridan, MIM, MBA, DHL

Director of Patient Engagement

PCORI Strategic Priorities for Engagement and Organizational Goals

- Development of PCOR Community
- Engagement in Research
- Overall Impact of PCORI – Influencing others to be more patient-centered

    - Ambassador Program
    - Pipeline to Proposal
    - Advisory Panel on Patient Engagement (PEAP)
    - Engagement Rubric
    - Engagement Officers
    - Compensation Framework
    - Current Patient/Caregiver Partners in PCORI Portfolio

Small Group Discussion Questions

- How do the engagement programs/activities map to the models for evaluation?

- What potential evaluation questions and metrics could we add to the evaluation models to more effectively demonstrate the value of the engagement program in:

    - Developing a PCOR community?
    - Engaging the community in research?
    - Influencing others to be more patient-centered?

Ambassador Program

Suzanne Schrandt, JD

Deputy Director of Patient Engagement

Emily Gagola

Program Associate

The Ambassador Program: Purpose

The Ambassador Program unites individual and organizational Ambassadors around the promise of patient-centered outcomes research (PCOR). The goal is to help patients, researchers, and other stakeholders, and their organizations, share PCORI's vision and mission with their communities, participate as full partners in research, and help ensure the sharing and use of information generated from PCORI-funded projects. The Ambassadors program can also serve as platform to facilitate shared learning between PCORI awardees, and to connect members of the PCOR community with opportunities in engagement across the healthcare spectrum.

The Ambassadors Program: Update

- Emily Gagola has taken over as new program lead January 25, 2016
- Currently 273 Ambassadors
- Ambassadors serving in a variety of functions;
    - American Medical Association-Convened Physician Consortium for Performance Improvement (PCPI)
    - An American College of Cardiology (ACC), American Geriatrics Society (AGS), and National Institute of Health (NIH) conference
    - Centers for Medicare & Medicaid Services (CMS) Technical Expert Panels on metrics for Qualified Health Plans
    - CMMI Technical Expert Panel developing performance measures for complex pediatric care
    - HHS Alzheimer's Advisory Council
    - MS Society, IOM Roundtables
    - CMS Partnership for Patients, NQF MAPP group

The Ambassador Program: Next Steps

- Launch public opening of program, targeting outreach to underrepresented groups
- Promote active involvement of currently or previously funded awardees in program
- Offer additional training/capacity building opportunities
- Enhance matchmaking mechanism
- Increase social media activity, compelling webinars and other events
- Develop Ambassador newsletter, created by Ambassadors

The Ambassadors Program: Metrics

- Numbers of Ambassadors
    - Including representation across racial and ethnic minority groups, geographic regions, and types of stakeholders
- Number of Ambassadors who’ve completed training
- Numbers and types of activities undertaken by Ambassadors (tool used is the “Activity Tracker”)
- Numbers and types of activities to which PCORI Ambassadors have been matched
- Number of PCORI awardees (science and engagement funding) who are Ambassadors
- Tracking of activity on Yammer (soon to be Chatter)

The Ambassador Program:
Small Group Discussion Questions

- How does the Ambassador Program map to the current evaluation models?

- What potential evaluation questions and metrics could we add to the evaluation models to more effectively demonstrate the value of the Ambassadors in:

    - Developing a PCOR community?
    - Engaging the community in research?
    - Influencing others to be more patient-centered?

Pipeline to Proposal Award Program

Courtney Clyatt, MA, MPH

Program Officer, Patient Engagement

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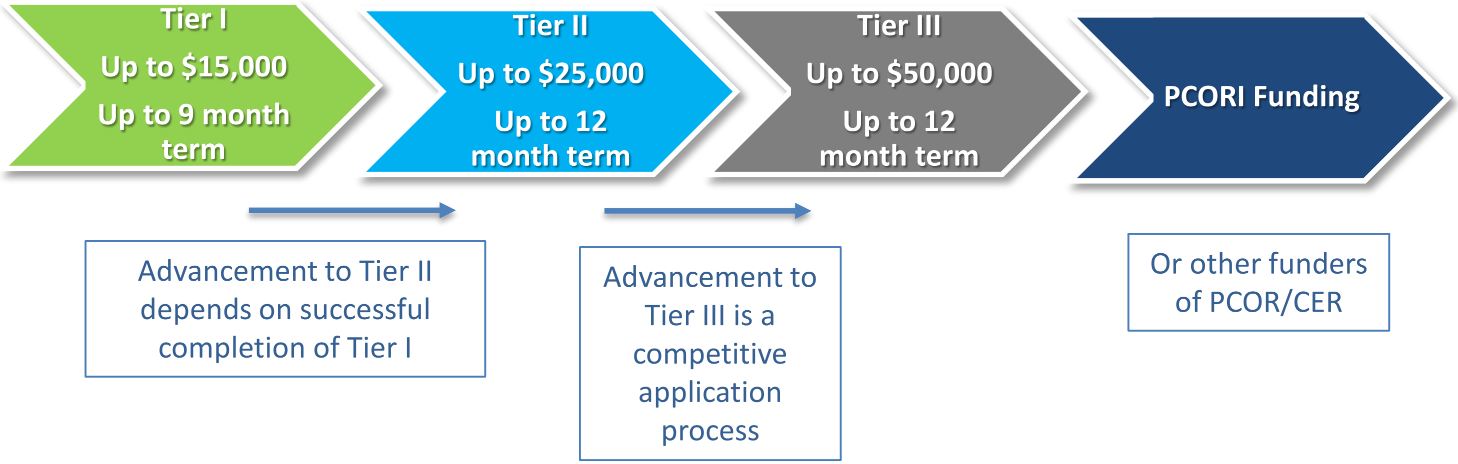
Pipeline to Proposal Award Program: Purpose



Tier I

Up to $15,000

Up to 9 month term

Tier II

Up to $25,000

Up to 12 month term

Tier III

Up to $50,000

Up to 12 month term

PCORI Funding

Advancement to Tier II depends on successful completion of Tier I

Advancement to Tier III is a competitive application process

Or other funders of PCOR/CER

Pipeline to Proposal Awards initiative aims to build a national community of patients, stakeholders, and researchers who have the expertise and passion to participate in patient-centered outcomes research, or PCOR, and to create partnerships within that community that lead to high-quality research proposals.

Pipeline to Proposal Award Program: Update

Our *Pipeline to Proposal Awards* encourage PCOR in comparative clinical effectiveness research.

Number of projects awarded:

Tier I – 77

Tier II – 27

Amount awarded:

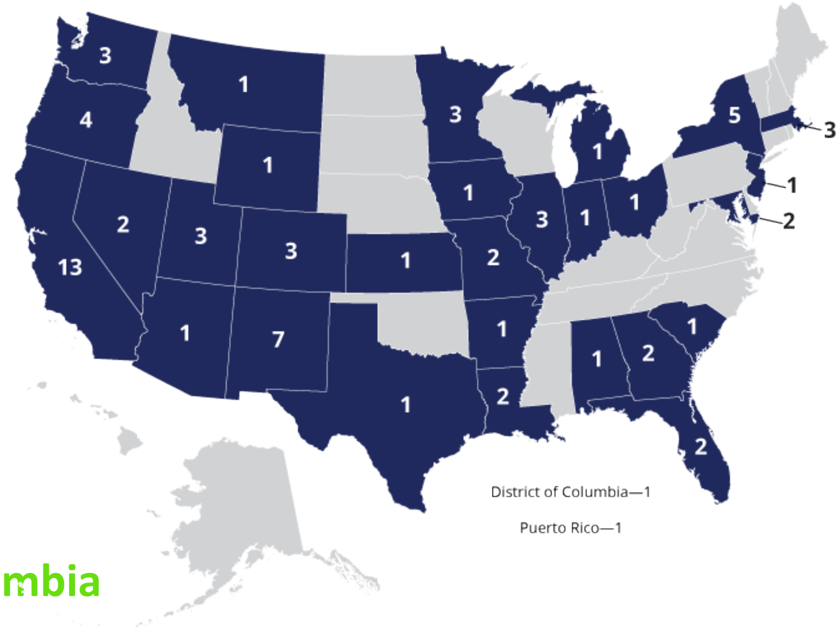
$1,813,999

(Tier I Cycles 1 & 2 and Tier II Cycle 1)

Number of states where we are funding projects:

30 states, District of Columbia and Puerto Rico

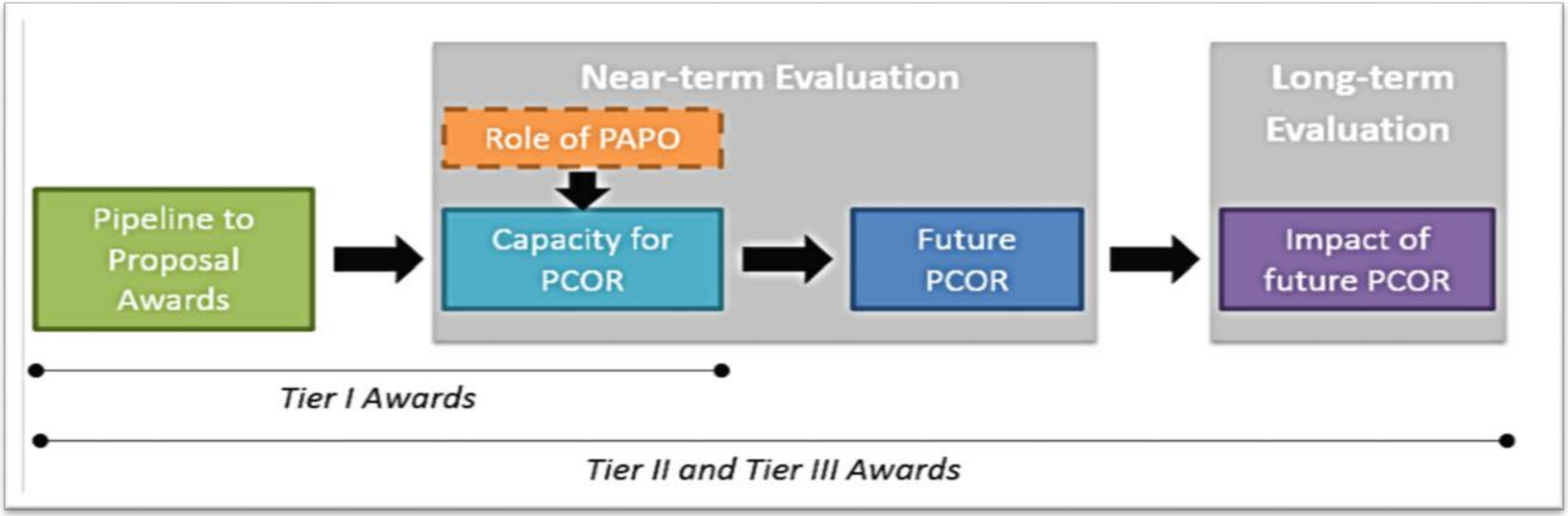
As of May 1, 2015



District of Columbia—1

Puerto Rico—1

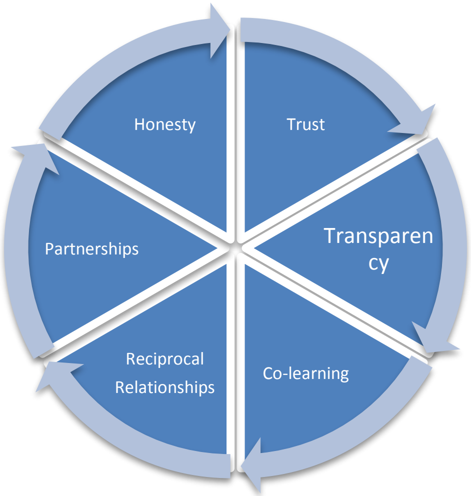
Evaluating the Pipeline to Proposal Awards



We hypothesize that Pipeline to Proposal Awards will **promote capacity for PCOR** (e.g., organizational structures, resources, collaborative relationships, policies, procedural protocols, and commitment to patient-centeredness needed to conduct PCOR). Moreover, we expect that this capacity will **lead to future PCOR** which will ultimately have a scientific and clinical impact.

Pipeline to Proposal Award Program: Metrics

- How many unique partnerships are being formed?
- What are some elements of successful partnership structures?
- How many awardees felt that the training and support they received prepared them to pursue research funding from PCORI or another funder?
- How many partnerships developed in the program embody the PCORI Engagement Principles?



Pipeline To Proposal Award Program: Small Group Discussion Questions

- How does the Pipeline to Proposal Award Program "map" to the current evaluation models?

- What potential evaluation questions and metrics could we add to the evaluation models to more effectively demonstrate the value of the Pipeline to Proposal Award Program in:

  - Developing a PCOR community?
  - Engaging the community in research?
  - Influencing others to be more patient-centered?

Advisory Panel on Patient Engagement (PEAP)

Sue Sheridan MIM, MBA, DHL

Director of Patient Engagement

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The Advisory Panel on Patient Engagement: Purpose

The purpose of the Advisory Panel on Patient Engagement is to ensure the highest patient engagement standards and a culture of patient-centeredness in all aspects of our work

The Advisory Panel on Patient Engagement: Update

Accomplishments

- Engagement Rubric
- Compensation Framework
- Participation at Annual Meeting
- Contributions to the PCORI Evaluation Group (PEG)
- Influencing others to be more patient centered in their work

The Advisory Panel on Patient Engagement: Next Steps

- Develop subcommittees on developing a training curriculum and engagement toolkit
- Help identify future measures of success and meaningful metrics for engagement
- Establish metrics to ensure diversity across all of PCORI engagement activities

The Advisory Panel on Patient Engagement: Metrics

- The number of tools developed and shared publicly to enhance PCOR as a result of input from PEAP members
- The number of PEAP members who have done something new to promote patient centeredness either within their respective organization or in other initiatives in which they are engaged
- The number of examples identified by PEAP members of PCORI's influence outside their organization in the broader healthcare eco-system

The Advisory Panel on Patient Engagement: Small Group Discussion Questions

- How does the PEAP map to the current evaluation models?

- What potential evaluation questions and metrics could we add to the evaluation models to more effectively demonstrate the value of the PEAP in:

    - Developing a PCOR community?
    - Engaging the community in research?
    - Influencing others to be more patient-centered?

Engagement Rubric

Suzanne Schrandt, JD

Deputy Director of Patient Engagement

Engagement Rubric: Purpose

The purpose of the Engagement Rubric is to provide a framework for meaningful engagement of patients and other stakeholders in the research process and to provide guidance to applicants, the patient/stakeholder community, awardees, merit reviewers, PCORI staff, and others in addressing engagement in research.

Engagement Rubric: Update

Version 3.0 is currently embedded in our funding announcements

Engagement language is incorporated into all PFAs, the engagement plan in each PFA is the mirror image of the rubric, and the rubric is used as a tool by applicants, merit reviewers, and awardees as well as by PCORI staff and other patients, stakeholders, and researchers

A CME developed specifically around the rubric has been posted on the website and has had 584 unique accesses and 70 certificates issued

Engagement Rubric: Next Steps

- Continue to evolve and refine the Engagement Rubric to reflect innovation in the field of engaged research

- Incorporate early findings regarding partnership development and "pre-engagement" strategies from the Pipeline to Proposals into the Engagement Rubric

- Work with Evaluation Team to determine the impact of the Engagement Rubric on the research community Including the patient/stakeholder partners

- Publish peer-reviewed journal article on the rubric

Engagement Rubric: Metrics

- The utility of the rubric in understanding application requirements is measured by the E&A team
- The utility of the rubric in conducting merit review is measured by the E&A team
- The use of the rubric in other healthcare sectors (beyond research)
- Incorporation of the rubric or references to the rubric by others
  - For example, it is used by the University of Alabama Birmingham to evaluate training for CTSAs
- Unique accesses and total certificates issued on the Engagement Rubric CME/CE

Engagement Rubric: Small Group Discussion Questions

- How does the Engagement Rubric “map” to the current evaluation models?

- What potential evaluation questions and metrics could we add to the evaluation models to more effectively demonstrate the value of the Engagement Rubric in:

  - Developing a PCOR community?
  - Engaging the community in research?
  - Influencing others to be more patient-centered?

Engagement Officers

Jaye Bea Smalley, MPA
Lisa Stewart, MA
Michelle Johnston-Fleece, MPH
Chinenye Anyanwu, PharmD, MPH

Patient Engagement Advisory Panel
April 11, 2016

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Engagement Officers: Purpose

Bridge to Science

- **Ensuring** adequate milestones, stakeholders and budgets
- **Supporting** funded research teams with engagement resources, tools and training
- **Monitoring** research project to ensure ongoing and meaningful engagement throughout the research project
- **Gathering** promising engagement practices and examples of impact of engagement in the research project
- **Sharing** with other Awardees and the broader research community

Engagement Officers: Update

Engagement Officers:

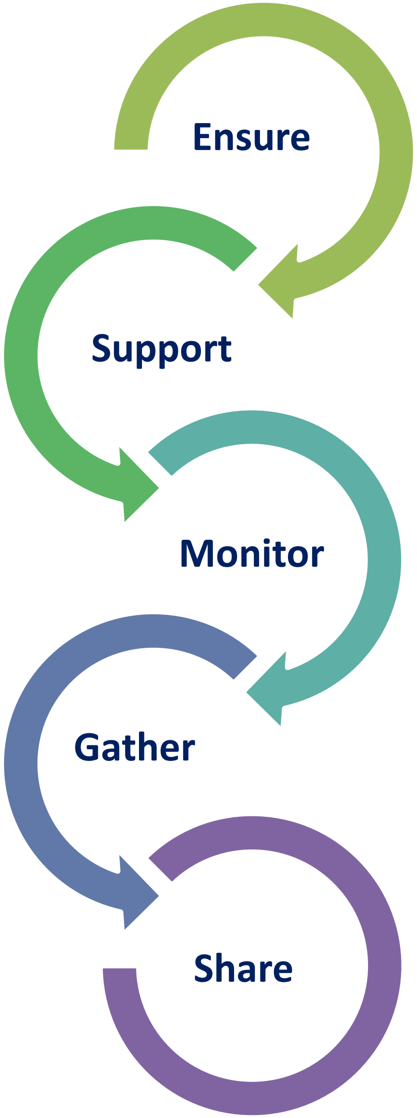
- Chinenye Anyanwu: Clinical Effectiveness Research
- Michelle Johnston-Fleece: Improving Healthcare Systems
- Jaye Bea Smalley: Methods and Infrastructure
- Lisa Stewart: Addressing Disparities

Refined approach:

- Focus on pragmatic clinical studies and targeted studies
- 44 PCS and targeted projects funded to date:
  - Appropriate patient/stakeholder organizational partnerships and budgets to support engagement plans
  - Attend in-person kick-off meetings inclusive of patient/stakeholders
- Consultative role for broad portfolio

Engagement Officers: Next Steps

- Develop new awardee cohort orientation
- Develop engagement webinar series to address needs of PCOR community and Awardees
- Author/co-author publications, blogs, abstracts; conduct presentations
- Contribute to national initiatives that promote engagement in research
- Develop and gather existing patient/stakeholder engagement trainings and tools
- Collect and archive promising practices from research portfolio
- Continue to monitor and support portfolio through consultations, kick-offs and site visits



Engagement Officer: Metrics

- Pre- and post-tests on webinars to assess learning of participants
- Number of external communications (presentations, blogs, publications, etc.) sharing promising engagement practices, impact of engagement and other knowledge
- Downloads of PCORI engagement tools and training materials

Engagement Officer: Small Group Discussion Questions

- How do the Engagement Officers map to the current evaluation models?

- What potential evaluation questions and metrics could we add to the evaluation models to more effectively demonstrate the value of the Engagement Officers in:

  - Developing a PCOR community?
  - Engaging the community in research?
  - Influencing others to be more patient-centered?

Compensation Framework

Charlotte Collins, JD

Chair, Advisory Panel on Patient Engagement

Suzanne Schrandt, JD

Deputy Director of Patient Engagement

The Compensation Framework: Purpose

The purpose of the Compensation Framework is to ensure fair financial compensation to patients, caregivers and patient/caregiver organizations who contribute time and effort to the planning, conduct and dissemination of research

The Compensation Framework: Update

- The Compensation Framework was first posted in June of 2015
- It is posted on the Engagement landing page as well as on the funding center page for any funding announcement
- Since posting the Compensation Framework, we have also updated the budgeting documents corresponding to the PFAs so that applicants are triggered to more appropriately budget for engagement
- Compensation Framework has been shared in various presentations and webinars
- We share the Compensation Framework with other agencies and organizations when they recruit Ambassadors and encourage others to compensate patients

The Compensation Framework: Next Steps

- Additional tool is being created so that our contracts administrators will be alerted when engagement is not budgeted appropriately

- Further analysis needed on our compensation data and trends

The Compensation Framework: Metrics

- Data on number and amount of compensation provided to patient partners across portfolio

- National Urban Fellow conducting analysis on trends in compensation and if it impacts level and success of engagement

- Uptake of compensation framework by other organizations engaging patients in their work

The Compensation Framework: Small Group Discussion Questions

- How does the Compensation Framework “map” to the current evaluation models?

- What potential evaluation questions and metrics could we add to the evaluation models to more effectively demonstrate the value of this framework in:

    - Developing a PCOR community?
    - Engaging the community in research?
    - Influencing others to be more patient-centered?

Current Patient/Caregiver Partners in the Research Portfolio

Sue Sheridan, MIM, MBA, DHL

Director of Patient Engagement

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Current Patient/Caregiver Partners in Research Portfolio: Purpose

Purpose:

- To influence research to be patient-centered, relevant, and useful
- To establish trust and a sense of legitimacy in research findings
- To encourage successful uptake and use of research results
- To share promising practices, barriers and facilitators, stories and perceptions of patient engagement in research

Current Patient/Caregiver Partners in Research Portfolio: Update

The total number of patients/caregivers involved (individuals and organizations)

- Research Portfolio—1226
- Approximately 310 of the 1226 are organizations

Current Patient/Caregiver Partners in Research Portfolio: Next Steps

- Continue training and capacity building:
    - Team science
    - PCOR/CER 101
    - Engagement Rubric training
    - Enroll in Ambassador community

- Collaborate with Evaluation Team and Dissemination Program area to mine portfolio to determine impact of engagement, promising practices, attitudes, facilitators and barriers, etc.

Current Patient/Caregiver Partners in Research Portfolio: Metrics

Current Efforts to Evaluate (and learn from) Engagement in Research

- WE-ENACT survey tool:
    - **Who** was engaged in your project (stakeholder communities represented),
    - **When** they were involved (which parts of the research process),
    - **How** they were engaged (what approaches you used and the level of engagement),
    - **How much** influence they had, and
    - **What** they did and what impact this had on the project
    - (Currently the PI submits survey and nomination of patient and/or stakeholder partners is voluntary)
- Interim Progress Reports
    - Submitted by PI
- Engagement Officers

Proposed Questions from the PEAP to Understand the Perspectives of the Engaged Partners

- Do you feel that your input was welcome?
- Do you feel that you had ample opportunity to present your views/opinions?
- Do you feel that your views/opinions was considered seriously?
- Do you feel that you received adequate training and support to participate effectively?
- Do you feel that you were treated as an equal by other members of the study team?

Proposed Questions from the PEAP to Understand the Perspectives of Engaged Partners

- Do you feel that the study team has adequately sought the input from you or other patient representatives (including patients, patient advocates and caregivers)?
- Do you feel that the study is designed to address the needs of patients?
- Do you feel that the way the study is being conducted is patient-centered?
- Do you feel that discussions/decisions about the study design were conducted with adequate transparency and respect?

Current Patient/Caregiver Partners in Research Portfolio: Small Group Discussion Questions

- How does the current patient/caregiver partner community map to the evaluation models?

- What potential evaluation questions and metrics could we add to the evaluation models to more effectively demonstrate the value of the patient/caregiver partner community in:

  - Developing a PCOR community?
  - Engaging the community in research?
  - Influencing others to be more patient-centered?

Small Group Breakouts #1

Main Conference Room

- **Model for Evaluating the Impact of PCORI: What is the Effect of PCORI's Approach to Developing a PCOR Community?**

- Charlotte W. Collins, JD (Chair)
- Regina Greer-Smith, MPH, FACHE
- Bruce L. Hanson, MDiv
- Lorraine Johnson, JD, MBA
- Julie Ginn Moretz, BS
- Sally Okun, BSN, RN, MMHS
- Kristin Carman, MA, PhD

Small Group Breakouts #2

Conference Room D

- **Model for Evaluating Engagement in Research: What is the Effect of Engagement in Research?**

- Jane Perlmutter, PhD, MBA
- Philip Posner, PhD
- Darius Tandon, PhD (Co-Chair)
- Veronica Todaro, MPH
- Amy L. Kratchman
- Jimmy Lin, MD, PhD, MHS
- Mark Mishra, MD

Small Group Breakouts #3

Huddle Room

- **Model for Evaluating the Overall Impact of PCORI: How Does PCORI's' Work Influence Others?**

- Steven I. Blum, MBA
- Marc Boutin, JD
- Anjum Khurshid, PhD
- Bennett Levitan, MD, PhD
- Kim McCleary
- Sara Traigle van Geertruyden, JD
- Perry Cohen, PhD

Report Out and Synthesizing Recommendations to PCORI Board of Governors' Evaluation Committee for Proposed New Evaluation and Measures on Engagement at PCORI

Salesforce Chatter

Rachel Melo

Executive Assistant

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Wrap-up, Next steps, and Reflections

Thank you

and

Safe Travels