Welcome

Please be seated by 8:35.

The webinar will go live at 8:45.

Patient-Centered Outcomes Research Institute

Assessment of Prevention, Diagnosis, and Treatment Options

Advisory Panel

September 21, 2013

Patient-Centered Outcomes Research Institute

Welcome: 8:45 am – 9:00 am

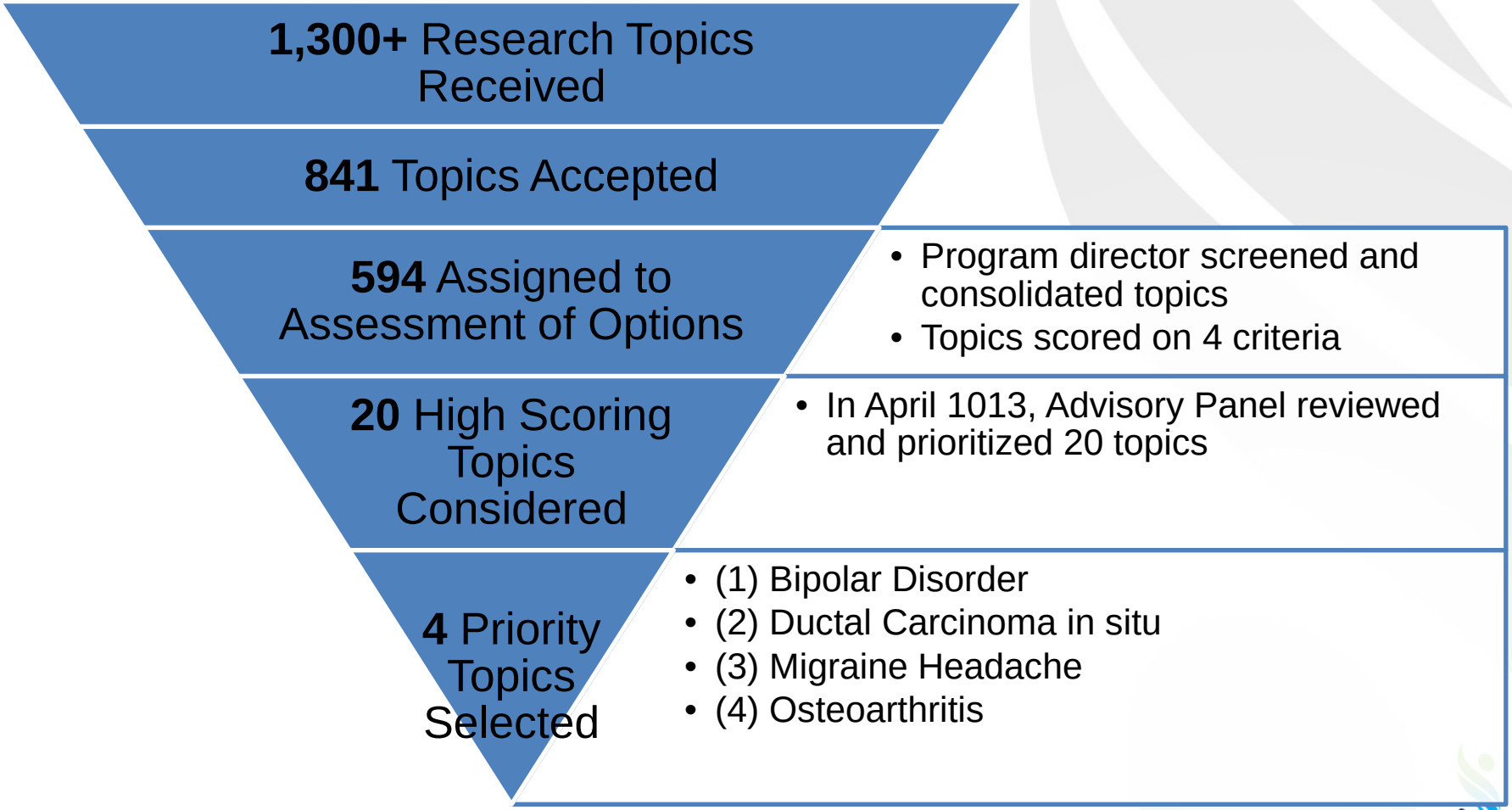
David Hickam, MD, MPH

Program Director
Clinical Effectiveness Research
PCORI

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
  - Comments may be submitted via email advisorypanels@pcori.org, no public comment period is scheduled
- For those in the room, please remember to speak loudly and clearly into a microphone
- Where possible, we encourage you to avoid technical language in your discussion

Background: Topic Prioritization



Meeting Objective

- Primary objective: further discuss the important research gaps for ductal carcinoma in situ and bipolar disorder and prioritize high priority research questions for both topics.

Moderators

Alvin I. Mushlin, MD, ScM

Chair, Panel on the Assessment of Options

Chairman, Department of Public Health, Weill Cornell Medical College; Public Health Physician-in-Chief, New York Presbyterian Hospital/Weill Cornell Medical Center

Margaret F. Clayton, RN, PhD

Co-chair, Panel on the Assessment of Options

Associate Professor, College of Nursing and Co-Director of the PhD Program, University of Utah

Agenda Overview

Time	Agenda Item
8:45-9:00 a.m.	Welcome and Overview of the Agenda
9:00-10:15 a.m.	Discussion of Key Research Questions for Ductal Carcinoma In Situ (DCIS)
10:15-10:30 a.m.	Break
10:30-11:45 a.m.	Discussion of Key Research Questions for Bipolar Disorder
11:45-12:00 p.m.	Review Voting Process for Research Question Prioritization
12:00-12:45 p.m.	Lunch
12:45-1:45 p.m.	Research Question Prioritization for DCIS & Bipolar Disorder
1:45-2:00 p.m.	Update on the Back Pain Targeted Funding Announcement
2:00-2:30 p.m.	Overview of the Assessment of Options Portfolio
2:30-3:00 p.m.	Strategic Approach of Advisory Panel
3:00-3:30 p.m.	Next Steps
3:30 p.m.	Adjourn

Advisory Panel Members

Topic Area Experts

Evan R. Myers, MD, MPH

Duke Evidence Synthesis Group

Matthew J. Crowley, MD

Duke Evidence Synthesis Group

Ductal Carcinoma In Situ (DCIS)
9:00 am – 10:15 am

Patient-Centered Outcomes Research Institute

Future Research Prioritization: Management Strategies for Ductal Carcinoma in Situ (DCIS)

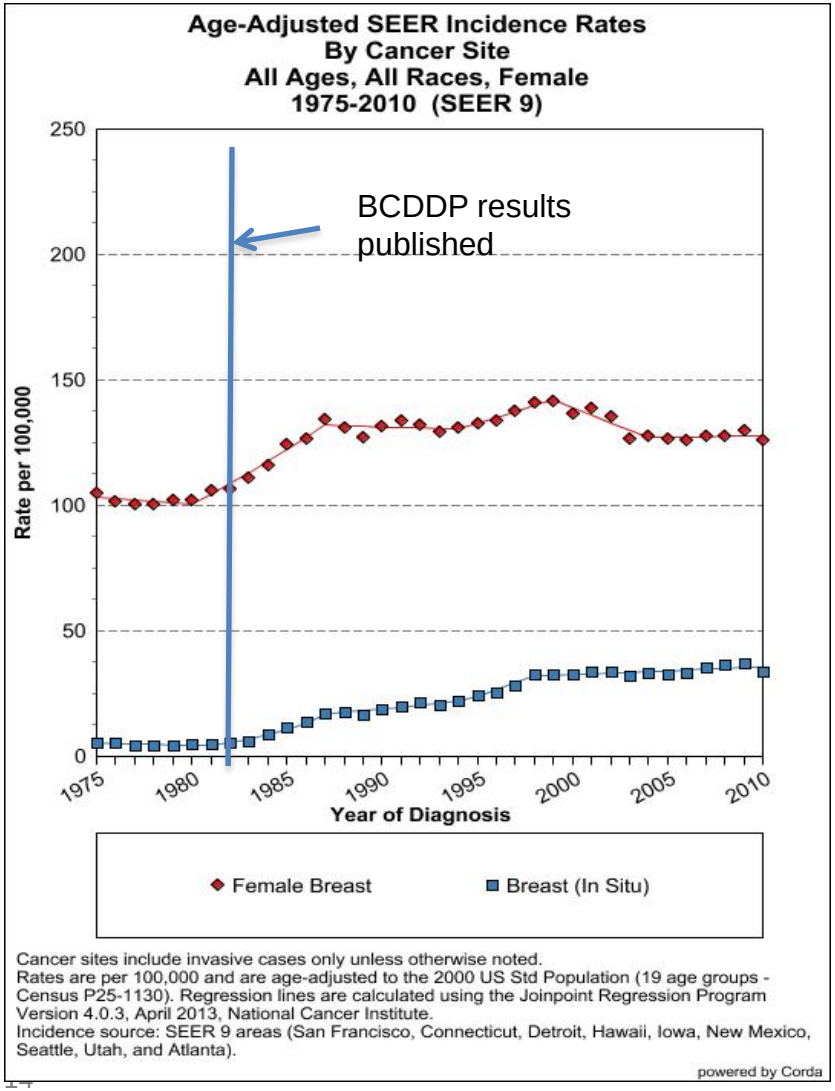
Evan R. Myers, MD, MPH

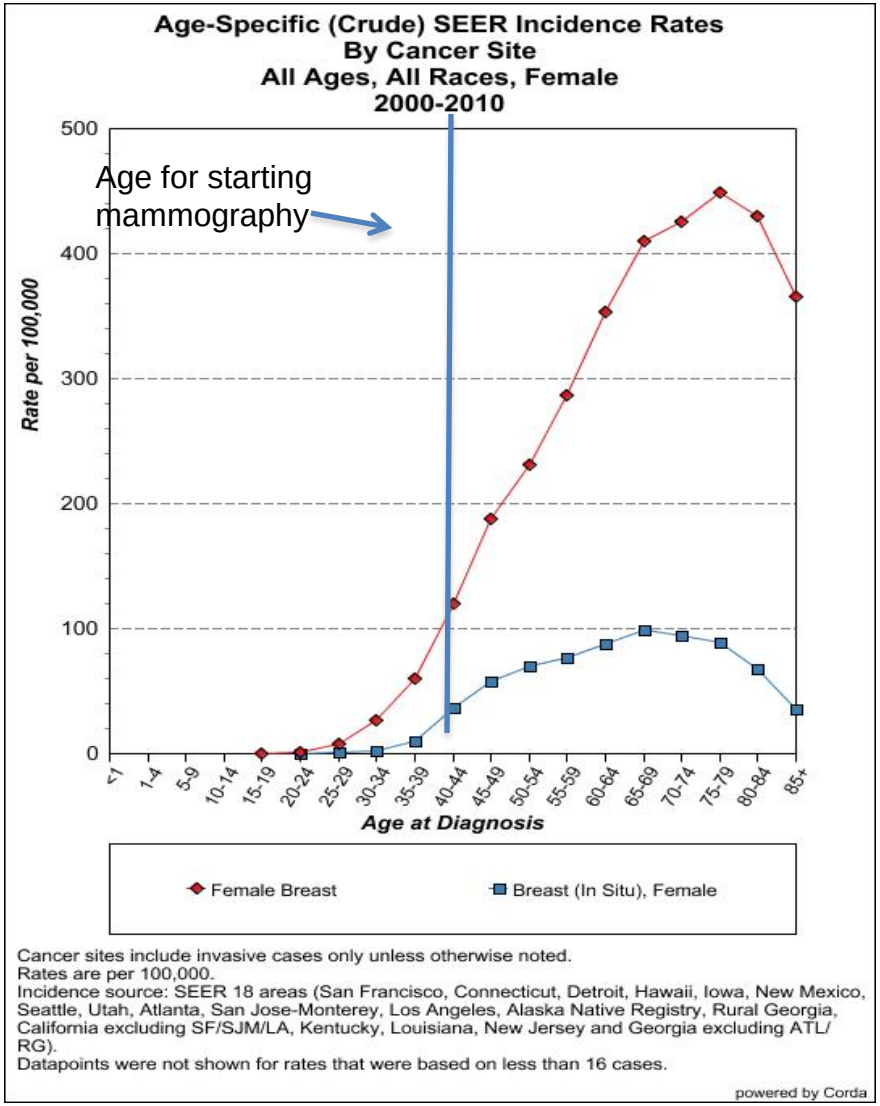
Duke Evidence Synthesis Group

Management Strategies for Ductal Carcinoma in Situ (DCIS)

- Ductal carcinoma in situ (DCIS):
  - Abnormal cells that look like cancer cells lining the milk ducts of the breast that have not invaded the underlying breast tissue
- DCIS is not cancer, but some cases will progress to invasive cancer.
- With increased use of mammography for breast cancer screening, the incidence of DCIS is rising.

Mammography and DCIS





Management Strategies for DCIS

- Main clinical issue:
  - No reliable way to predict which patients with DCIS will go on to develop invasive cancer and which will not
- Long-term survival is almost 100% with all current treatment options.
- Various treatment options for DCIS present trade-offs relevant to patient-centered outcomes such as symptoms, function, and well-being.

Overall Project Goal

- To work with stakeholders to help PCORI **identify, refine, and prioritize future research evidence gaps** in the area of management strategies for Ductal Carcinoma in Situ (DCIS)

Overview of Project

1. Identifying Known Evidence Gaps
2. Creation of Stakeholder Group
3. Expansion of Evidence Gaps
4. Analytic Framework
5. Stakeholder Prioritization
6. Horizon Scan
7. Study Design Considerations

Identifying Known Evidence Gaps

- Review of published systematic reviews, clinical practice guidelines, and future research needs documents
- Initial 20 evidence gaps explored:
  - Specific **populations** or subgroups of patients
  - Comparative safety and effectiveness of available **interventions and comparators**
  - Impact of treatment of specific **outcomes** of interest
  - Optimal **timing** or **setting** for treatment

Creation of Stakeholder Group

- American Cancer Society
- American College of Surgeons
- American Society of Breast Surgeons
- American Society of Clinical Oncology
- American Society of Therapeutic Radiology and Oncology
- Centers for Disease Control and Prevention (CDC), National Breast and Cervical Cancer Early Detection Program
- Cancer Prevention and Treatment Fund
- National Breast Cancer Coalition
- National Cancer Institute
- Society of Surgical Oncology
- Patient Advocate

Stakeholders

Peter D. Beitsch, MD
President, Dallas Surgical Group
Director, Dallas Breast Center

Laura J. Esserman, MD, MBA
Professor, Surgery & Radiology
University of California, San Francisco

Temeika L. Fairley, PhD
Lead Health Scientist
Centers for Disease Control and Prevention, National Center for Chronic Disease Prevention and Health Promotion, Division of Cancer Prevention and Control

Brandel France de Braavo
Director of Public Affairs and Communications
Cancer Prevention and Treatment Fund/National Research Center For Women & Families

Worta McCaskill-Stevens, MD, MS
Chief, Community Oncology and Prevention Trials Research Group
National Cancer Institute

Donna Pinto
Patient Advocate

Rinaa S. Punglia, MD, MPH
Assistant Professor, Department of Radiation Oncology
Harvard Medical School

Joy Simha
Member of Board of Directors, Young Survival Coalition

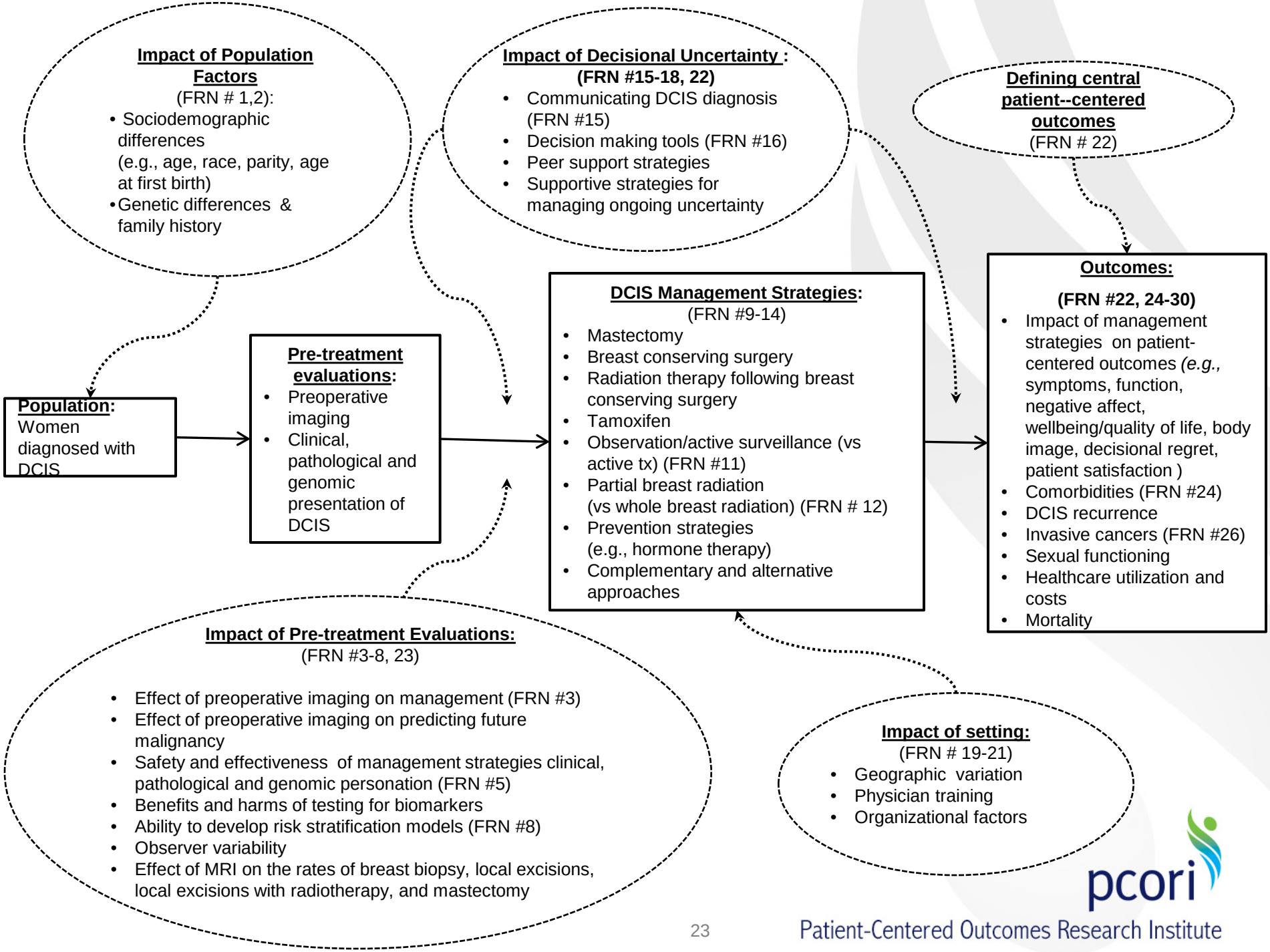
Debbie Saslow, PhD
Director, Breast and Gynecologic Cancer
Department of Cancer Control Science
American Cancer Society

Stakeholder Discussion Themes

- Considerable uncertainty about diagnosis, prognosis, and treatment
- Variability in provider recommendations
- Potential patient distress both during initial diagnosis and treatment – and after treatment
- Need for studies exploring:
  - comparative studies of techniques to improve diagnostic and prognostic certainty
  - decision support interventions to manage uncertainty
  - outcomes of an active surveillance strategy to more conventional treatment options
- Need to incorporate broader range of patient-centered outcomes into studies

Future Research Needs Broad Topic Areas Covering 30 Questions

1. Sociodemographic differences, genetics, family history
2. Imaging, pathology, biomarkers, observer variability
3. Risk stratification
4. Management strategies including observation, drugs, radiotherapy, surgery
5. Decision-making, communication, support
6. Modifiers – geography, physician specialty, organizational factors
7. Patient-centered outcomes (e.g., symptoms, well-being, sexual functioning) and other outcomes like costs to patients



Stakeholder Prioritization

- Online ranking of evidence gaps
- Forced-ranking prioritization method
  - 10 votes per stakeholder, which could be allocated to any of the 30 research priorities
  - Maximum of 3 votes per item
- Asked to rank based on “most important unanswered research question in the management strategies of DCIS”
- Questions divided into a top, middle, and lower tier
- Only top tier moved on to final stage of horizon scan and study design considerations

Stakeholder Prioritization – TOP TIER

Question	Score	N
Is it possible to develop and validate **risk stratification models** to accurately identify women with DCIS for whom specific management strategies are preferred?	9	7
What is the comparative safety and effectiveness of **observation/active surveillance versus immediate treatment**?	8	6
Do comparative safety and effectiveness of management strategies depend on **clinical, pathological, and genomic presentations** of DCIS?	7	5
What is the comparative effectiveness of different approaches to **communicating the diagnosis** of DCIS to the patient?	7	6
What is the comparative effectiveness of **decision making tools**?	7	6
What are the comparative sensitivity and specificity of **preoperative imaging evaluations for detecting occult invasive breast cancer** among DCIS patients?	5	4
What is the impact of DCIS management strategies on **comorbidities**?	5	3
What are the comparative safety and effectiveness of **partial breast radiation therapy versus whole breast radiation therapy**?	4	5
What are the most important patient-centered outcomes for women diagnosed with DCIS?	4	2
What is the impact of DCIS management strategies on **invasive cancers**?	4	3

Horizon Scan

- PubMed
  - 1417 articles identified
  - 105 included as potentially relevant to top tier questions
  - 15 systematic reviews, 3 RCTs, 83 cohort studies, 1 case-controlled study, and 3 modeling studies
  - Sample sizes ranged from 11 to 23,547 patients
- ClinicalTrials.gov
  - 206 protocols identified
  - 37 included as potentially relevant to top tier questions
  - 12 RCTs, 5 observational studies, 20 non-randomized interventional trials
  - Sample sizes ranged from 2 to 4300 patients

Horizon Scan Summary

Question	SRs	RCTs	Cohort	Case Control	Model	Ongoing
Risk stratification models	2	0	12	1	1	0
Active surveillance	1	0	3	0	0	0
Predictors of outcomes	7	0	26	1	1	3
Communicating diagnosis	0	0	1	0	0	0
Decision making tools	0	0	0	0	0	1
Preoperative imaging	1	0	12	0	0	5
Impact on comorbidities	2	0	4	0	0	19
Partial/whole radiation	2	0	7	0	0	24
Most important PC outcomes	0	0	0	0	0	0
Impact on invasive cancers	11	3	30	0	2	9

Study Design Suggestions

Question	RCT	Meta Analysis		Observational		Model
		RCTs	Observational	New Data	Existing Data	
Risk stratification models	No	Yes	?	?	Yes	?
Active surveillance	?	No	No	Yes	?	?
Predictors of outcomes	No	Yes	?	?	Yes	?
Communicating diagnosis	Yes	?	No	?	?	No
Decision making tools	Yes	?	No	?	?	No
Preoperative imaging	?	?	?	Yes	Yes	?
Impact on comorbidities	Yes	?	?	?	?	?
Partial/whole radiation	Yes	No	?	?	?	?
Most important PC outcomes	No	No	No	Yes	?	No
Impact on invasive cancers	?	?	?	?	?	?

Summary: Stakeholder Priorities

- Identifying most important outcomes
  - Prerequisite to addressing any of the other priorities
    - No published or ongoing studies
- Resolving uncertainty about outcomes
  - Methods for estimating likely outcomes of different choices for individual patients
    - Clinical/pathological/radiological/genomic predictors/models
    - Comparative effectiveness of specific diagnostic and treatment options (including observation)
      - Area with most published literature, ongoing research
- Managing uncertainty about outcomes
  - Methods for communicating diagnosis, management options
  - Methods for facilitating decision making
    - No published papers, 1 ongoing study

Questions and Discussion

Discussion: Research Gaps in Ductal Carcinoma In Situ (DCIS)

- How well do the research questions meet the 5 PCORI criteria?
  - Patient-Centeredness
  - Impact of the Condition on the Health of Individuals and Populations
  - Options for Addressing the Issue
  - Likelihood of Implementation in Practice
  - Durability of Information

Break
10:15 am – 10:30 am

Patient-Centered Outcomes Research Institute

pcori

Bipolar Disorder
10:30 am – 11:45 pm

Patient-Centered Outcomes Research Institute

Future Research Prioritization: Bipolar Disorder and Antipsychotic Use in Adolescents and Young Adults

Matthew J. Crowley, MD

Duke Evidence Synthesis Group

Bipolar Disorder and Antipsychotic Use in Adolescents and Young Adults

- Bipolar disorder, commonly called “manic depression,” is a serious brain disorder that causes extreme and unusual changes in moods and behaviors
- Bipolar disorder that starts in childhood or the early teen years seems to be more severe than when the disorder starts in older teens and adults
- There is no cure for bipolar disorder
- Antipsychotic medications are used to control symptoms of bipolar disorder and provide symptom relief in adolescents and young adults (defined as youths under 25 years of age). However, antipsychotics may carry significant side effects.

Overall Project Goal

- To work with stakeholders to help PCORI **identify, refine, and prioritize future research evidence gaps** in the area of bipolar disorder and antipsychotic use in adolescents and young adults

Overview of Project

1. Identifying Known Evidence Gaps
2. Creation of Stakeholder Group
3. Expansion of Evidence Gaps
4. Analytic Framework
5. Stakeholder Prioritization
6. Horizon Scan
7. Study Design Considerations

Identifying Known Evidence Gaps

- Review of published systematic reviews, clinical practice guidelines, and future research needs documents
- Initial 21 evidence gaps explored:
  - Specific **populations** or subgroups of patients
  - Comparative safety and effectiveness of available **interventions and comparators**
  - Impact of treatment of specific **outcomes** of interest
  - Optimal **timing** or **setting** for treatment

Creation of Stakeholder Group

- American Academy of Child and Adolescent Psychiatrists
- American Academy of Pediatrics
- American Psychiatric Association
- American Society of Clinical Psychopharmacology
- National Federation of Families for Children's Mental Health
- Patient Advocate

Stakeholders

Robert B. Christian, M.D.
Assistant Professor of Psychiatry and Pediatrics
University of North Carolina

Christoph U. Correll, M.D.
Associate Professor of Psychiatry and Molecular Medicine
Zucker Hillside Hospital

Laura J. Fochtmann, M.D.
Professor, Department of Psychiatry and Behavioral Science
Stony Brook University School of Medicine

Martha S. Gerrity, M.D., M.P.H., Ph.D.
Professor
Division of Hospital and Specialty Medicine/Section of General Medicine
Portland VA Medical Center

Teresa King
Training & TA Family Resource Specialist
National Federation of Families for Children's Mental Health and the Georgetown University National Technical Assistance Center for Children's Mental Health

Marvin J. Sawyer Jr., M.S., L.P.C., N.C.C.
Youth Involvement Content Specialist and Community Technical Assistance Lead
National Federation of Families for Children's Mental Health

Thomas Scott Stroup, M.D., M.P.H.
Professor of Psychiatry
Columbia University College of Physicians and Surgeons
Research Psychiatrist, New York State Psychiatric Institute

Monica C. Wehby, M.D.
Pediatric Specialist, Neurosurgery
Legacy Medical Group
Patient Advocate

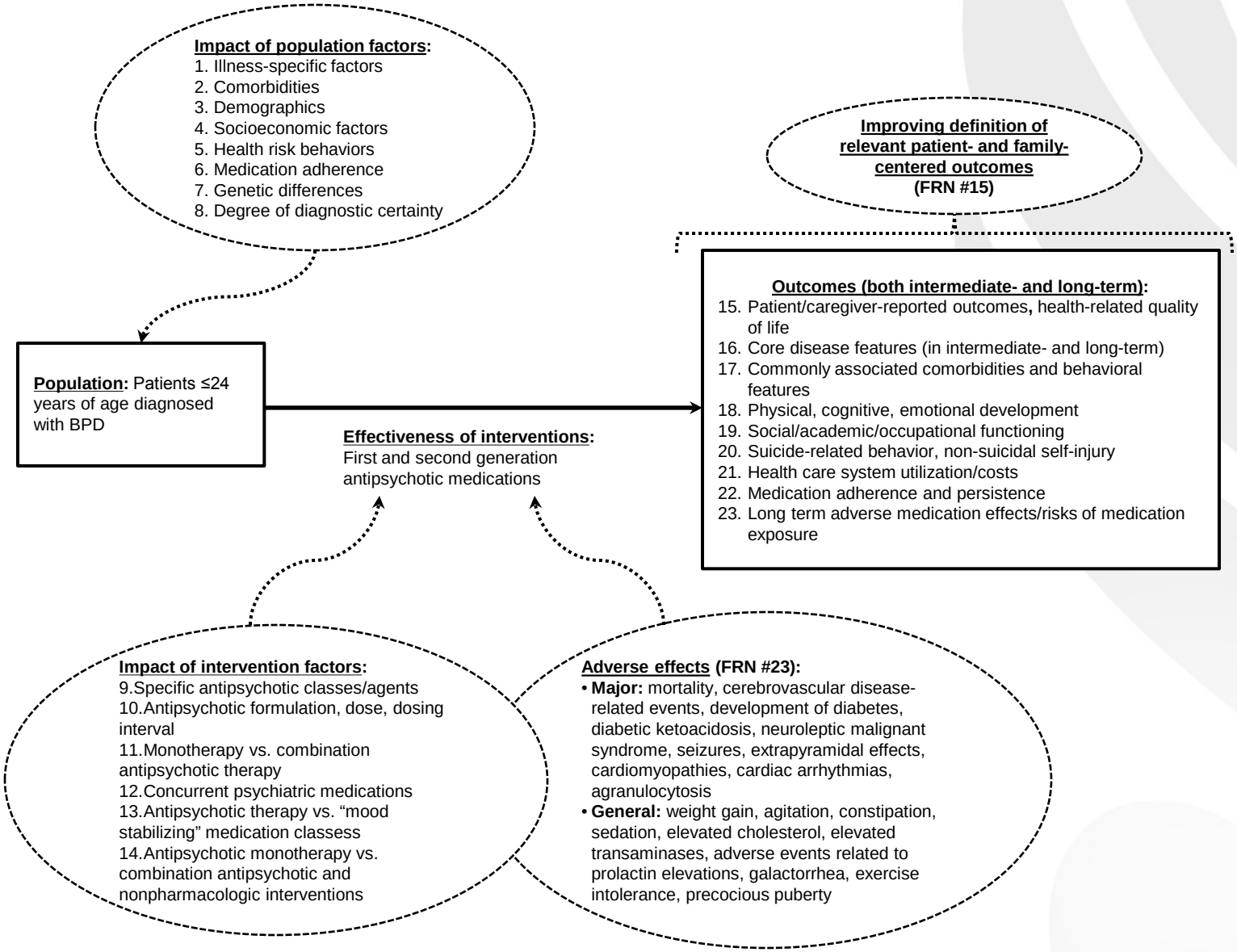
Julie M. Zito, Ph.D.
Professor of Pharmacy and Psychiatry
Department of Pharmaceutical Health Services Research
University of Maryland School of Pharmacy

Stakeholder Discussion Themes

- Need to consider broad set of patient-centered outcomes including:
  - Outcomes over longer time frames
  - Functional status outcomes
  - Developmental outcomes
  - Outcomes related to parents, caregivers or family members that include economic outcomes
  - Outcomes related to offspring in women of childbearing years
- Efficacy of antipsychotic drug treatment compared with alternative drug classes is still uncertain for many subgroups of these young patients
- Role and comparative safety and effectiveness of concomitant therapies

Future Research Needs Broad Topic Areas Covering 23 Questions

1. Socioeconomic and demographic factors
2. Illness-specific or genetic factors
3. Psychiatric, medical, and neurodevelopmental comborbidities
4. Impact of health-risk behaviors
5. Medication adherence and adverse effects
6. Treatment strategies including specific classes/agents, concurrent medications, nonpharmacologic interventions
7. Patient-centered outcomes (e.g., core disease features, behavioral features, physical/cognitive/emotional development, social/academic/occupational functioning)


Impact of population factors:
1. Illness-specific factors
2. Comorbidities
3. Demographics
4. Socioeconomic factors
5. Health risk behaviors
6. Medication adherence
7. Genetic differences
8. Degree of diagnostic certainty
Improving definition of relevant patient- and family-centered outcomes (FRN #15)
Outcomes (both intermediate- and long-term):
15. Patient/caregiver-reported outcomes, health-related quality of life
16. Core disease features (in intermediate- and long-term)
17. Commonly associated comorbidities and behavioral features
18. Physical, cognitive, emotional development
19. Social/academic/occupational functioning
20. Suicide-related behavior, non-suicidal self-injury
21. Health care system utilization/costs
22. Medication adherence and persistence
23. Long term adverse medication effects/risks of medication exposure
Population: Patients ≤24 years of age diagnosed with BPD
Effectiveness of interventions:
First and second generation antipsychotic medications
Impact of intervention factors:
9.Specific antipsychotic classes/agents
10.Antipsychotic formulation, dose, dosing interval
11.Monotherapy vs. combination antipsychotic therapy
12.Concurrent psychiatric medications
13.Antipsychotic therapy vs. "mood stabilizing" medication classess
14.Antipsychotic monotherapy vs. combination antipsychotic and nonpharmacologic interventions
Adverse effects (FRN #23):
• Major: mortality, cerebrovascular disease-related events, development of diabetes, diabetic ketoacidosis, neuroleptic malignant syndrome, seizures, extrapyramidal effects, cardiomyopathies, cardiac arrhythmias, agranulocytosis
• General: weight gain, agitation, constipation, sedation, elevated cholesterol, elevated transaminases, adverse events related to prolactin elevations, galactorrhea, exercise intolerance, precocious puberty

Stakeholder Prioritization

- Online ranking of evidence gaps
- Forced-ranking prioritization method
  - 10 votes per stakeholder, which could be allocated to any of the 23 research priorities
  - Maximum of 3 votes per item
- Asked to rank based on “most important unanswered research question in the management strategies of bipolar disorder and antipsychotic use in adolescents and young adults”
- Questions divided into a top, middle, and lower tier
- Only top tier moved on to final stage of horizon scan and study design considerations

Stakeholder Prioritization – TOP TIER

Question	Score	N
What are the comparative safety and effectiveness of **monotherapy compared to combination therapy**?	12	6
What are the comparative effects of antipsychotics on **social, academic, and occupational functioning**?	9	6
What are the **key patient-centered and family-centered outcomes,** and how are these outcomes affected by different antipsychotic classes/agents?	8	5
What are the comparative safety and effectiveness of **concurrent psychiatric medications** (e.g., antidepressants, anxiolytics, stimulants) given as adjuncts ?	7	5
What are the comparative safety and effectiveness of **"mood stabilizing" medication classes** (e.g., lithium or antiepileptic drugs like lamotrigine or valproic acid)?	7	5
What are the comparative safety and effectiveness of antipsychotic drugs alone compared with the **combination of antipsychotic drugs plus other nonpharmacologic interventions**?	6	4
What are the **adverse effects of short-term and long-term medication exposure**?	5	5
How do the comparative safety and effectiveness of antipsychotic treatment differ depending on **demographic differences**?	4	3
How do the comparative safety and effectiveness of antipsychotic treatment differ depending on **socioeconomic factors**?	4	3
What are the comparative effects of antipsychotics on **core disease features**?	4	4

Horizon Scan

- PubMed
  - 1563 articles found in original search
  - 42 included as potentially relevant to the top tier questions
  - 20 RCTs, 15 cohort studies, 1 case-control study, and 6 systematic reviews/meta-analyses
  - Sample size: 12 to 8129 patients

- ClinicalTrials.gov
  - 95 active protocols
  - 42 included as potentially relevant to the top tier questions
  - 30 RCTs, 1 observational study, 11 nonrandomized intervention trial
  - Sample size: 13 to 5000 patients

Horizon Scan Summary

Question	SRs	RCTs	Cohort	Case Control	Model	Ongoing
Monotherapy vs. combination	1	0	1	0	0	7
Social, academic, occupational functioning	1	7	4	0	0	0
Key patient- and family-centered outcomes	2	3	3	1	0	3
Concurrent medications	0	1	1	0	0	3
Mood stabilizing medications	1	5	3	0	0	4
Nonpharmacologic interventions	0	3	0	1	0	3
Medication adverse effects	5	9	7	0	0	14
Demographic differences	0	0	2	0	0	1
Socioeconomic factors	0	0	0	0	0	0
Core disease features	3	7	5	0	0	22

Study Design Suggestions

Question	RCT	Meta Analysis		Observational		Model
		RCTs	Observational	New Data	Existing Data	
Monotherapy vs. combination	Yes	?	?	?	Yes	?
Social, academic, occupational functioning	Yes	?	No	Yes	?	No
Key patient- and family-centered outcomes	No	No	No	Yes	?	No
Concurrent medications	Yes	?	?	?	Yes	?
Mood stabilizing medications	Yes	?	?	?	Yes	?
Nonpharmacologic interventions	Yes	?	?	Yes	Yes	?
Medication adverse effects	Yes	?	?	Yes	Yes	?
Demographic differences	No	?	Yes	Yes	Yes	?
Socioeconomic factors	No	?	Yes	Yes	Yes	?
Core disease features	Yes	Yes	?	?	Yes	?

Summary: Stakeholder Priorities

- Identifying most important outcomes
  - Prerequisite to addressing any of the other priorities
    - Existing studies on short-term adverse effects
    - Studies on long-term adverse effects and identification of key patient- and family-centered outcomes scarce
- Comparative safety and effectiveness of specific interventions
  - Comparative studies which evaluate diverse treatment regimens studying both short- and long-term patient-centered outcomes of interest are needed
- Impact of socioeconomic/demographic patient factors
  - Least studied and highlight the need for future research

Questions and Discussion

Discussion: Research Gaps in Bipolar Disorder

- How well does the research question meet the 5 PCORI criteria?
  - Patient-Centeredness
  - Impact of the Condition on the Health of Individuals and Populations
  - Options for Addressing the Issue
  - Likelihood of Implementation in Practice
  - Durability of Information

Review Voting Process for Research Question Prioritization: 11:45 am – 12:00 pm

- Forced-ranking prioritization method
  - For each topic, 5 votes per person, which could be allocated to any of the 10 research priorities
  - Maximum of 3 votes per item
- Rankings should be completed during lunch

Lunch
12:00 pm – 12:45 pm

Patient-Centered Outcomes Research Institute

Research Question Prioritization for DCIS & Bipolar Disorder
12:45 pm – 1:45 pm

Patient-Centered Outcomes Research Institute

Research Question Ranking for DCIS

- Combine 8 and 5: Vote for 8.
- Combine 24, 11, and 26: Vote for 11.
- Combine 15 and 16: Vote for 15.
- Remaining single topics:
  - 3
  - 12
  - 22

Research Question Ranking for Bipolar Disorder

- Combine 11, 13, and 9. Vote for 11.
- Combine 19 and 15. Vote for 19.
- Combine 3 and 4. Vote for 3.
- Remain as single topics:
  - 12
  - 14
  - 23
  - 16

Update on the Back Pain Targeted Funding Announcement: 1:45 pm – 2:00 pm

Regina Dehen, ND, LAc

Member, PCORI Treatment Options for Back Pain Task Force

Chief Medical Officer, National College of Natural Medicine Clinic

Harold Sox, MD

Member, PCORI Treatment Options for Back Pain Task Force

Professor of Medicine (Emeritus, Active), Geisel School of Medicine at Dartmouth and The Dartmouth Institute for Health Policy and Clinical Practice

Background: Targeted Funding for Back Pain

December 2012

- Five topics, including Treatment Options for Back Pain, selected for potential targeted funding

March 2013

- Back Pain Workgroup reached consensus on five broad research gaps

July – August 2013

- Task force narrowed research gaps

Research Gap Areas Identified by PCORI Workgroup (March 2013)

1. Classification and categorization of back pain causation and symptomatology
2. Comparisons of strategies of care
3. Choice of outcomes for evaluating treatments
4. Psychosocial factors that affect outcomes for non-specific back pain
5. Decision support and education of health care providers

Back Pain Task Force

- In July 2013 a task force was formed to provide expert input in narrowing the broad research gaps identified by the previous workgroup into a concise list of well-defined high priority research questions for potential PCORI funding.

- The task force met in July and August and reviewed the findings of the ad hoc workgroup and an updated literature review.

Back Pain Task Force Conclusions

- Refined list of broad research gaps to identify the most important issues.
- Conclusion: The highest priority issue is predicting/preventing the transition from acute to chronic back pain.
  - Testing a comprehensive multi-modal approach for managing episodes of acute low back pain
  - Identifying psychosocial and other predictors of transition from acute to chronic/relapsing low back pain and developing a clinical prediction rule for this transition.

Back Pain Targeted Funding Next Steps

- PCORI staff to decide whether or not to move forward with a targeted funding announcement

- If we decide to move forward, a draft funding announcement will be circulated among a subset of the back pain task force for feedback

Overview of the Assessment of Options Portfolio: 2:00 pm – 2:30 pm

Diane Bild, MD, MPH

Senior Program Officer
Clinical Effectiveness Research
PCORI

Questions addressed

- What are the characteristics of the studies we have funded?
- How do we measure success in our program?
- How do we modify our policies, procedures, and PFAs to achieve our goals?

Reminder of Program Timeline

Key Dates			
Action	**Cycle I**	**Cycle II**	**Cycle III****
PFA Release Date	May 22, 2012	N/A	N/A
Online System Opening Date	June 1, 2012	September 15, 2012	January 15, 2013
Letter of Intent* (LOI) Due Date	June 15, 2012	October 15, 2012	February 15, 2013
Informational Webinars (Specific dates to be posted on pcori.org.)	June and July 2012	October – November 2012	February – March 2013
Application Deadline	July 31, 2012	November 30, 2012	March 31, 2013
Merit Review Dates	August – November 2012	December 2012 – March 2013	April – July 2013
Awards Announced	December 31, 2012	April 2013	August 2013
Earliest Start Date	January 2013	May 2013	September 2013

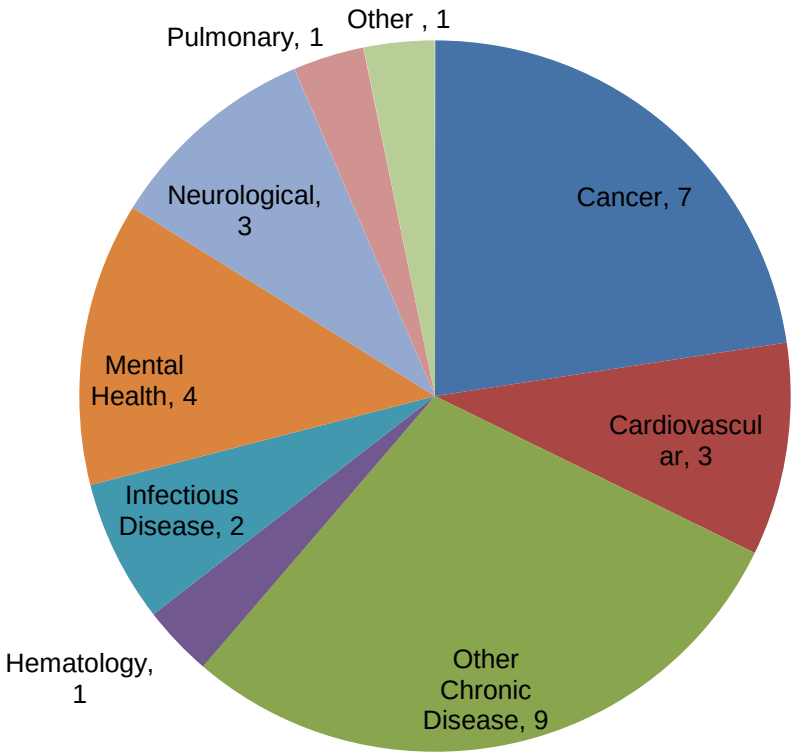
Source: http://pcori.org/assets/PFA-Assessment-of-Options-052220121.pdf

PFA and slate characteristics

Cycle/Submission date	Cycle I	Cycle II	Cycle III	Aug 2013
Approx. award date	January 2013	July 2013	September 2013	January 2014
Maximum budget/yr (direct costs)	$500K	$500K	$500K	$500K
Max project period	3 years	3 years	3 years	3 years
Funds available	$32M	$34M	$48M	$32M
# review criteria	8	8	8	5
# awards made	9	22	24	TBD
Total funding	$53.1M		$45.7M	TBD

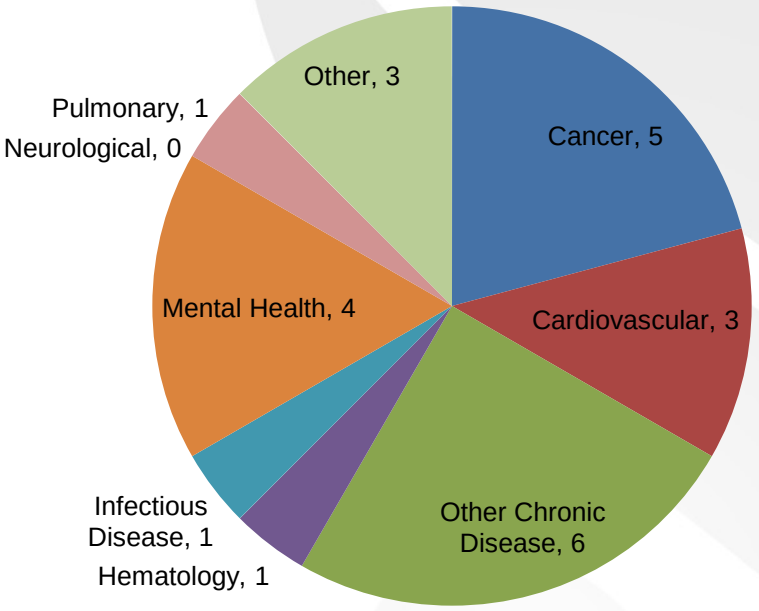
Diseases & Conditions – Cycles I & II and III

Cycles I & II



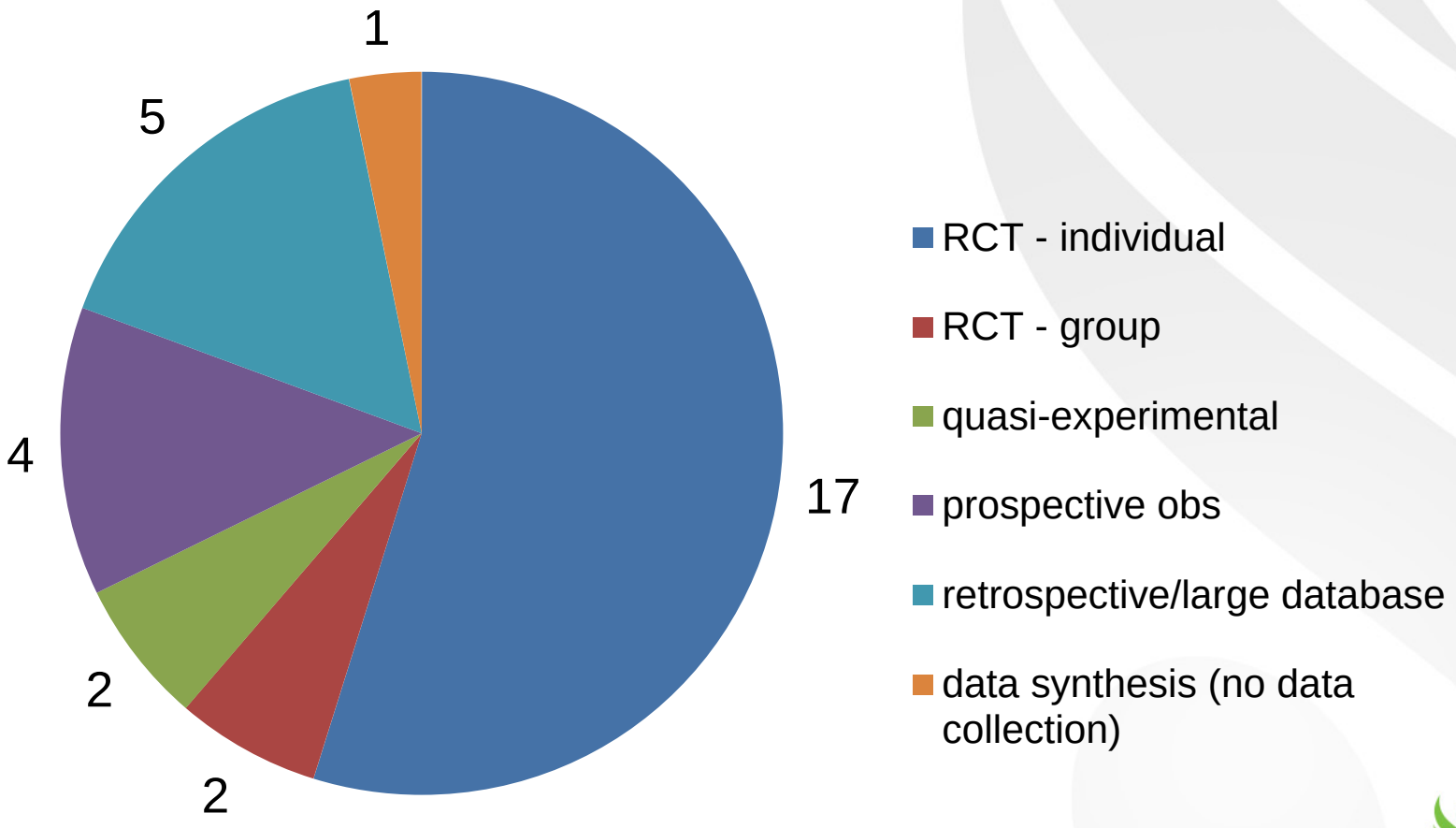
31 projects

Cycle III



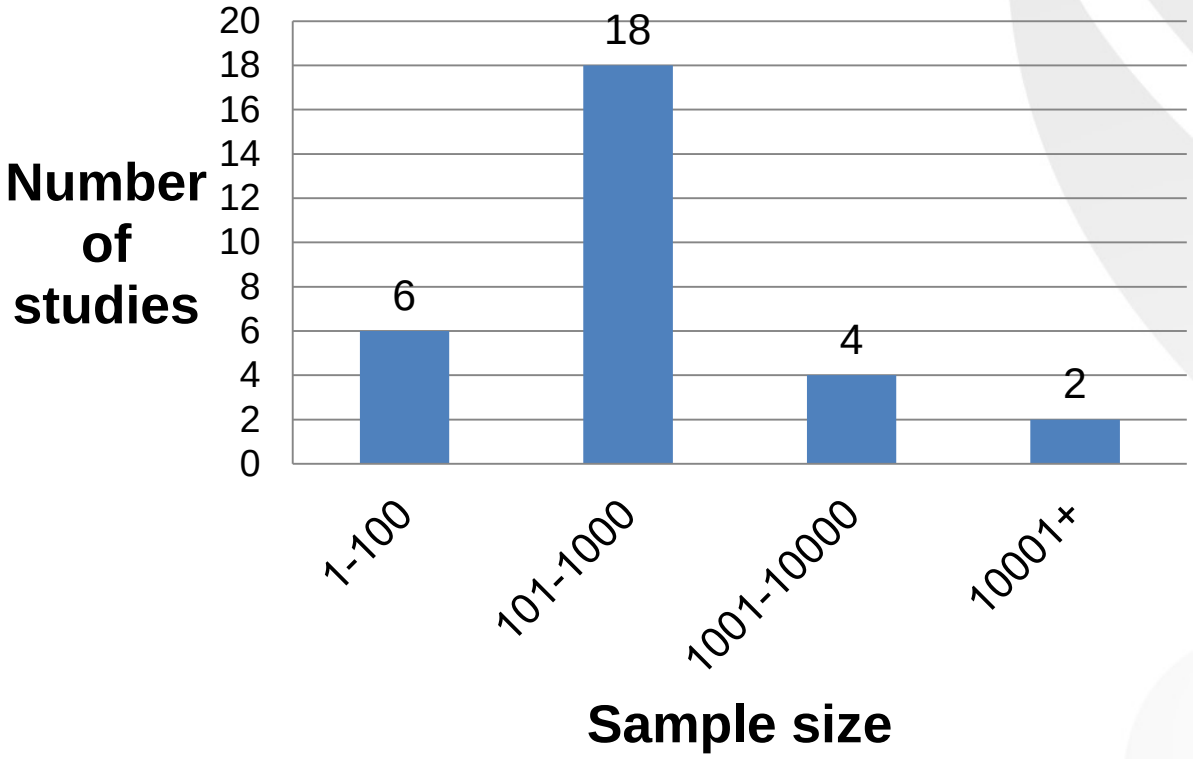
24 projects

Study design for main CER analysis – Cycle I & II projects



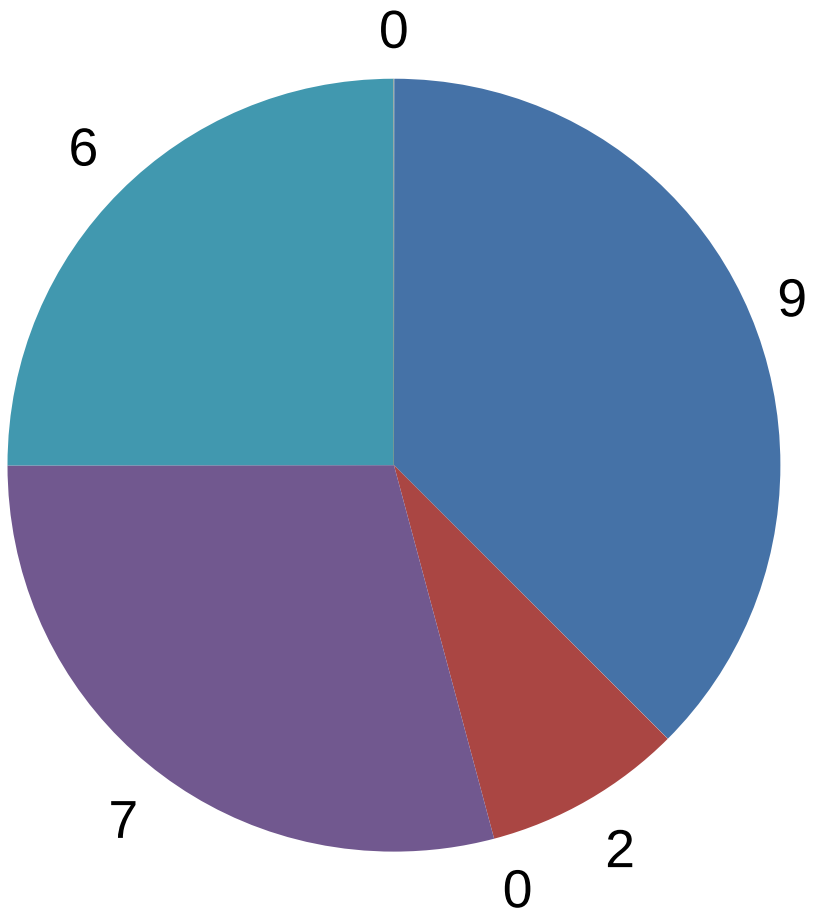
31 projects, CER Program

Sample Size for main CER analysis, Cycles I & II



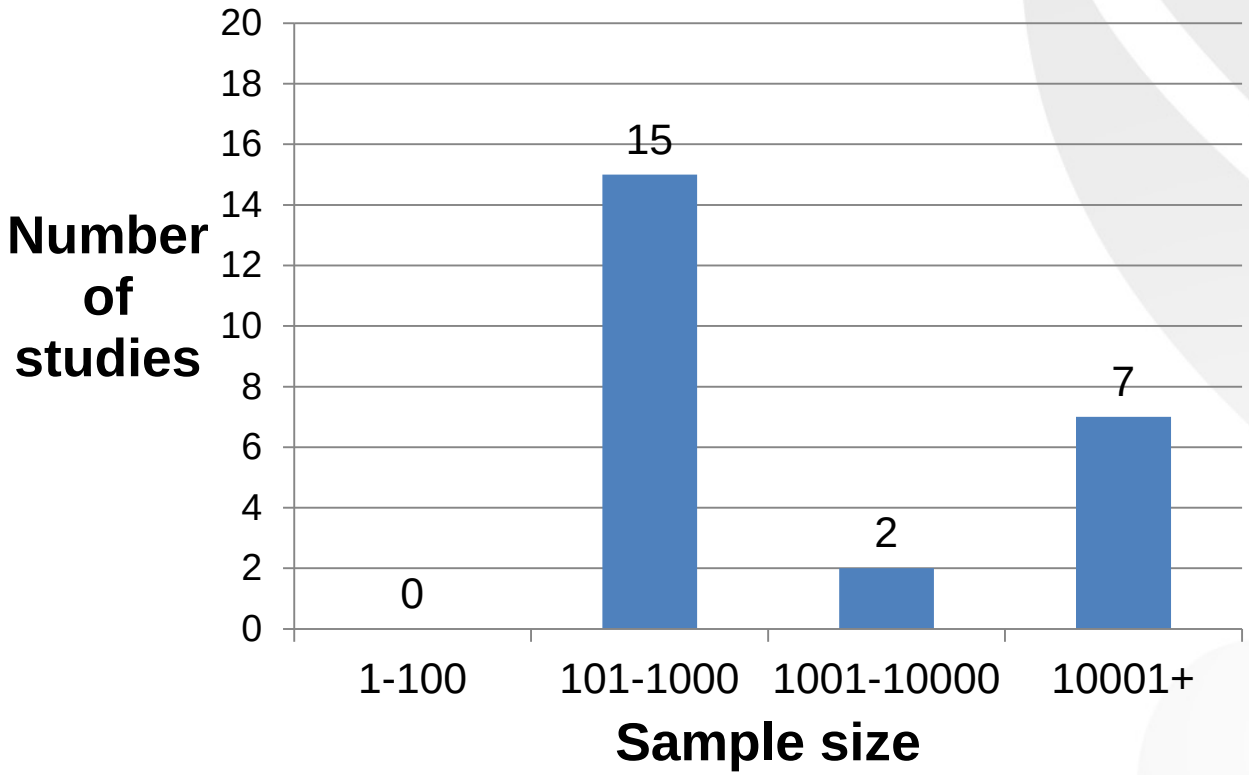
One study not included because it is not collecting primary data.

Study design for main CER analysis – Cycle III projects



24 projects, CER Program

Sample Size for main CER analysis, Cycle III



Priority populations included

- Racial and ethnic minority groups
- Low-income groups
- Women
- Children (age 0–17)
- Older adults (age 65 and older)
- Residents of rural areas
- Individuals with special healthcare needs, including individuals with disabilities
- Individuals with multiple chronic diseases
- Individuals with rare diseases
- Individuals whose genetic make-up affects their medical outcomes
- Patients with low health literacy/numeracy and limited English proficiency
- Lesbian, gay, bisexual, transsexual (LGBT) persons

Proposed Vision Statement for CER Program

- To encourage and manage a set of timely, high quality, impactful, unique, comparative effectiveness studies that are balanced across topic areas. The studies' findings will be useful to patients and decision makers and encounter minimal barriers in implementation.

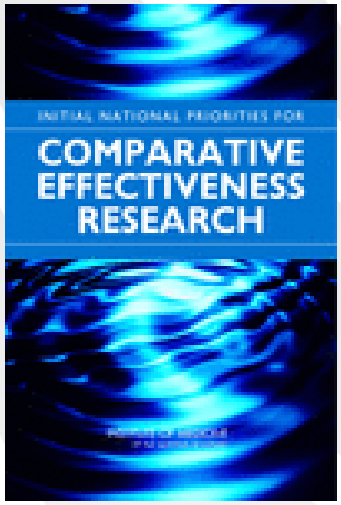
Charting a course for CER

- “PCORI should identify specific high-priority research questions, perhaps using the Institute of Medicine list as a starting point.”

- Hal Sox

Sox H. The Patient-Centered Outcomes Research Institute should focus on high-impact problems that can be solved quickly. Health Affairs 2012; 31: 2176–2182.

Mapping CER Portfolio to IOM CER Priorities – Cycles I, II & III



2009

- Among the 55 projects:
  - 6 are closely related
    - 4 -- 1st quartile
    - 1 -- 2nd quartile
    - 1 -- 4th quartile
  - 23 are somewhat related
    - 5 -- 1st quartile
    - 13 -- 2nd quartile → 10 fall into a priority on SMDM or DA
    - 3 -- 3rd quartile
    - 2 -- 4th quartile
  - 26 are unrelated

SMDM=shared medical decision-making; DA=decision aide
One project was deemed to fall into 2 IOM categories.

Other external benchmarks

- America's Health Insurance Plans research priorities
- PCORI solicitations of CER questions
- Global Burden of Disease
- AHRQ systematic reviews
- Review of clinical guidelines

http://www.who.int/topics/global_burden_of_disease/en/

Summary and Conclusions

- CER portfolio includes a wide range of conditions, populations, and study designs; would like to encourage large RCTs, inclusion of priority populations
- Modifying PFAs to encourage further improvement
  - Encourage clinical trials
  - Issue targeted PFAs
    - Fibroids
    - DCIS
    - Bipolar in children
    - Back pain
- Welcome suggestions on
  - This presentation – how to portray the portfolio
  - How to encourage more impactful research

Questions and Discussion

Strategic Approach of Advisory Panel: 2:30 pm – 3:00 pm

- Potential new topics
  - Lung cancer
  - Genetic testing for cancer
- Strategic approach
  - Single conditions versus themes across conditions (e.g. chronic pain, compliance with oral drug regimens, rare diseases)
  - Topics that advance the methodology of comparative effectiveness research

Next Steps: 3:00 pm – 3:30 pm

- Next in-person meeting tentatively scheduled for Monday, January 13th – Tuesday, January 14th in Washington, DC (location TBD)
  - Discuss and prioritize research gaps for migraine headache and osteoarthritis
  - Prioritize new research topics

pcori

Thank you for your participation.

Patient-Centered Outcomes Research Institute