



# **PCORI Advisory Panels**

## **Winter 2015 Meetings**

### **General Session**

Arlington, VA

January 13-14 2015

Patient-Centered Outcomes Research Institute

# Advisory Panels Here Today

- Assessment of Prevention, Diagnosis, and Treatment Options
- Addressing Disparities
- Patient Engagement
- Rare Disease

# Three-Day Agenda

Start Time	Item
Tuesday, January 13, 2015	
8:30 a.m.	<p>General Session:</p> <ul style="list-style-type: none"><li>• PCORI's Funding Strategy – B. Luce</li><li>• Evaluating PCORI's Approach to Topic Generation, Prioritization, and Selection – L. Forsythe</li></ul>
9:15 a.m.	Break
9:30 a.m.	<p>Individual Panel Meetings:</p> <ul style="list-style-type: none"><li>• Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options (Crystal Ballroom Salon C)</li><li>• Advisory Panel on Addressing Disparities (Lincoln Hall – Lower Level)</li><li>• Advisory Panel on Patient Engagement (Crystal Ballroom Salon B)</li><li>• Advisory Panel on Rare Disease (Pentagon 1 &amp; 2)</li></ul>

# Three-Day Agenda (cont.)

Start Time	Item
12:00/12:30 p.m.	Lunch
1:00/1:30 p.m.	Individual Panel Meetings
6:00 p.m.	Reception (Crystal Ballroom Salon A)
6:30 p.m.	Dinner (Crystal Ballroom Salon A)
Wednesday, January 14, 2015	
8:00 a.m.	Breakfast (Crystal Ballroom Salon A)
8:30 a.m.	General Session: <ul style="list-style-type: none"><li>• PCORI's Funding Strategy – B. Luce</li><li>• Evaluating PCORI's Approach to Topic Generation, Prioritization, and Selection – L. Forsythe</li></ul>
9:15 a.m.	Break

# Three-Day Agenda (cont.)

Start Time	Item
9:30 a.m.	Individual Panel Meetings <ul style="list-style-type: none"><li>• Advisory Panel on Improving Healthcare Systems (Crystal Ballroom Salon C)</li><li>• Advisory Panel on Patient Engagement (Crystal Ballroom Salon B)</li><li>• Advisory Panel on Clinical Trials (Pentagon 1 &amp; 2)</li></ul>
12:00/12:30 p.m.	Lunch
1:00/1:30 p.m.	Individual Panel Meetings
6:00 p.m.	Reception (Van Buren Room)
6:30 p.m.	Dinner (Van Buren Room)
Thursday, January 15, 2015	
8:00 a.m.	Breakfast (Crystal Ballroom Salon A)
8:30 a.m.	Individual Panel Meeting: <ul style="list-style-type: none"><li>• Advisory Panel on Improving Healthcare Systems (Crystal Ballroom Salon C)</li></ul>

# Advisory Panel Responsibilities

## Standing Topic Priority-Setting Advisory Panels:

- APDTO
- IHS
- AD

→ Evaluate and recommend priority topics for consideration in PFAs

## Patient Engagement: Helps PCORI ensure the highest patient engagement standards and a culture of patient-centeredness in all aspects of our work

## Clinical Trials: Advises PCORI regarding the selection, research design, implementation, and technical issues of clinical trials for PCOR

## Rare Disease: Advises PCORI regarding the conduct of patient-centered CER in rare diseases and the coordination and engagement with the rare disease research community





# PCORI's 5-Year Research Funding Strategy

Bryan Luce, PhD, MBA  
Chief Science Officer, PCORI

Patient-Centered Outcomes Research Institute

# Funding Vision

- Continue but taper broad research program
  - Continue to evaluate and build upon existing portfolio to identify and strategically manage clusters of research projects
- Continue to target key topics
- Continue Large Pragmatic Clinical Studies program
- Prepare and operationalize PCORnet for CER research

# Specific Issues To Improve Funding Process (1)

## Topic priority-setting process

- More in-depth topic briefs
- Better identification of specific researchable questions

## “Usual Care” as a comparator

- Definition?
- An appropriate comparator? If so, when?
  - Should UC policy differ for CER vs IHS, AD studies?
- Should UC be standardized? E.g. “best practice”
- How to analyze and disseminate findings?

# Specific Issues To Improve Funding Process (2)

## Improving methodological rigor

- Methodological consultation post merit review (MC, CTAP)
- Complex/pragmatic designs (MC, CTAP)
- Methodological challenges in rare disease research (RDAP, CTAP)

## Improving *conduct* of funded trials

- Recruitment/retention guidelines (CTAP)
- Ongoing methodological review/support (MC, CTAP)

## Special concerns for research on rare diseases

- Increasing funding opportunities
- Evaluating merit review criteria



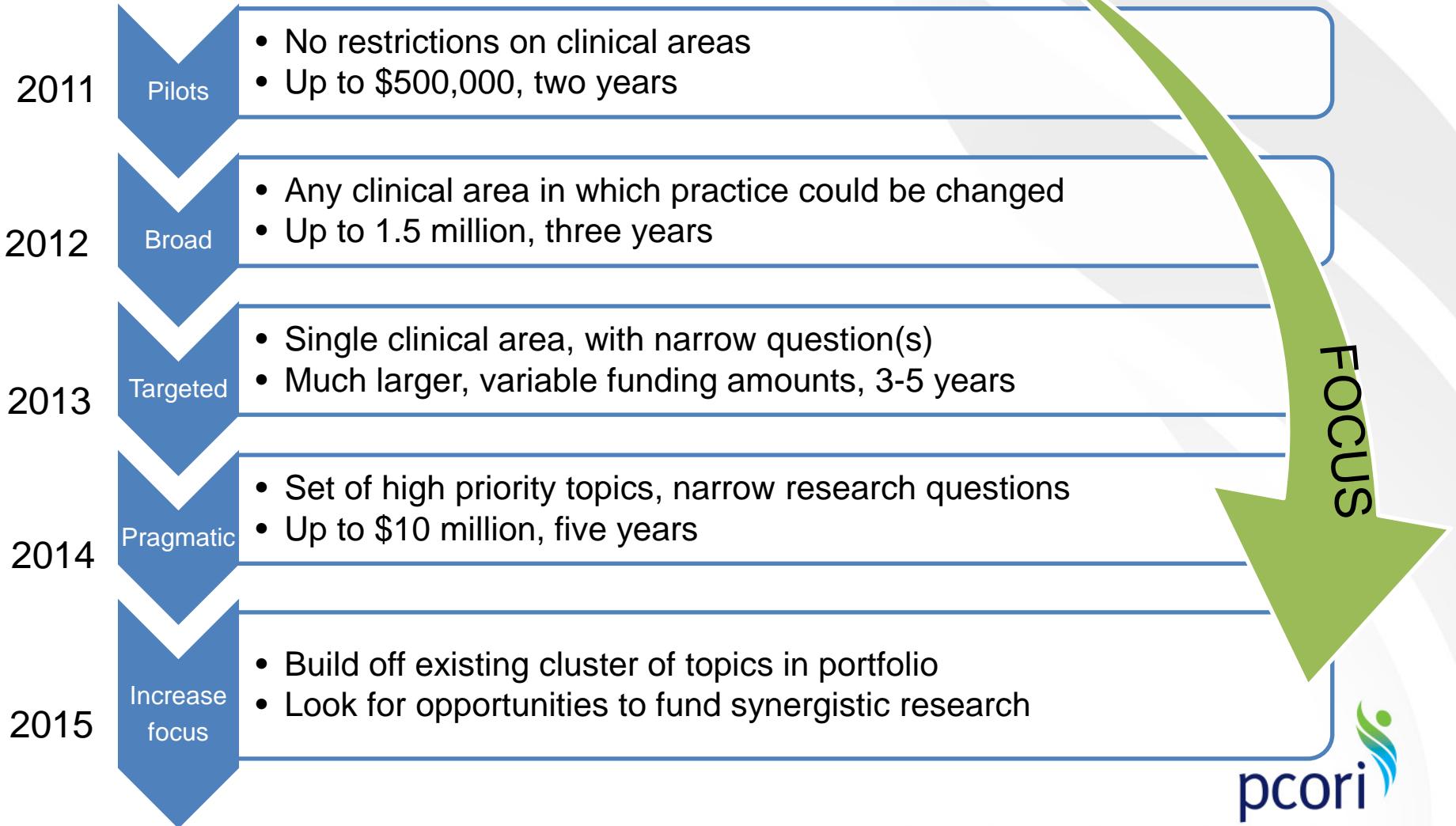
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# Specific Issues To Improve Funding Process (3)

- Growing interest in “cross-cutting” health CER, e.g. syndromes, symptoms (pain, anxiety)
  - Generally
  - Rare diseases (RDAP)
- Improving HealthCare Systems CER
  - Active Collaborating with AHRQ
  - Evaluating appropriate comparator designs (e.g. “usual care”)
- Funding CER in PCORnet
  - Proof of concept studies: RCT, observational

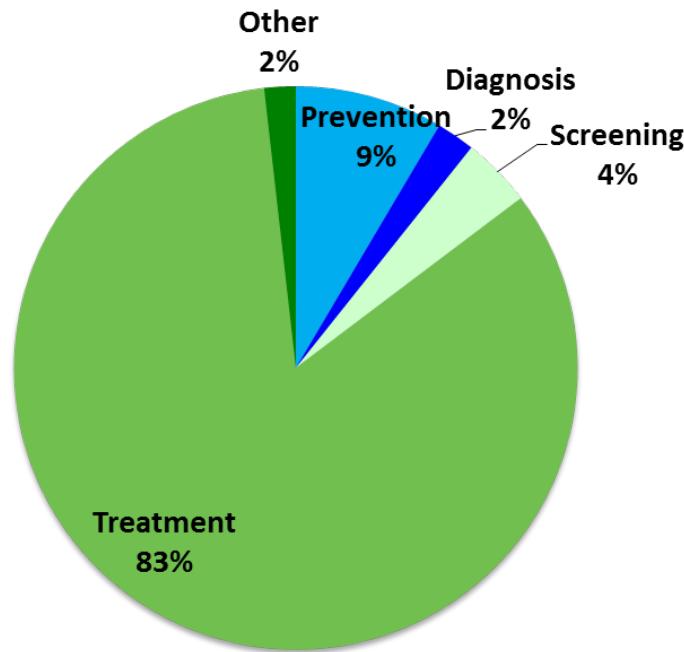


# Increasing Focused Research Funding



# Snapshot of Current Portfolio

# Funded Projects by Care Continuum Proportion of Total (n=223)\* and Award Amount (n=\$492M)



**223 Studies  
in Total**

*(All funded cycles to  
date, including  
targeted, not  
including Methods)*

**\$492M in Total**

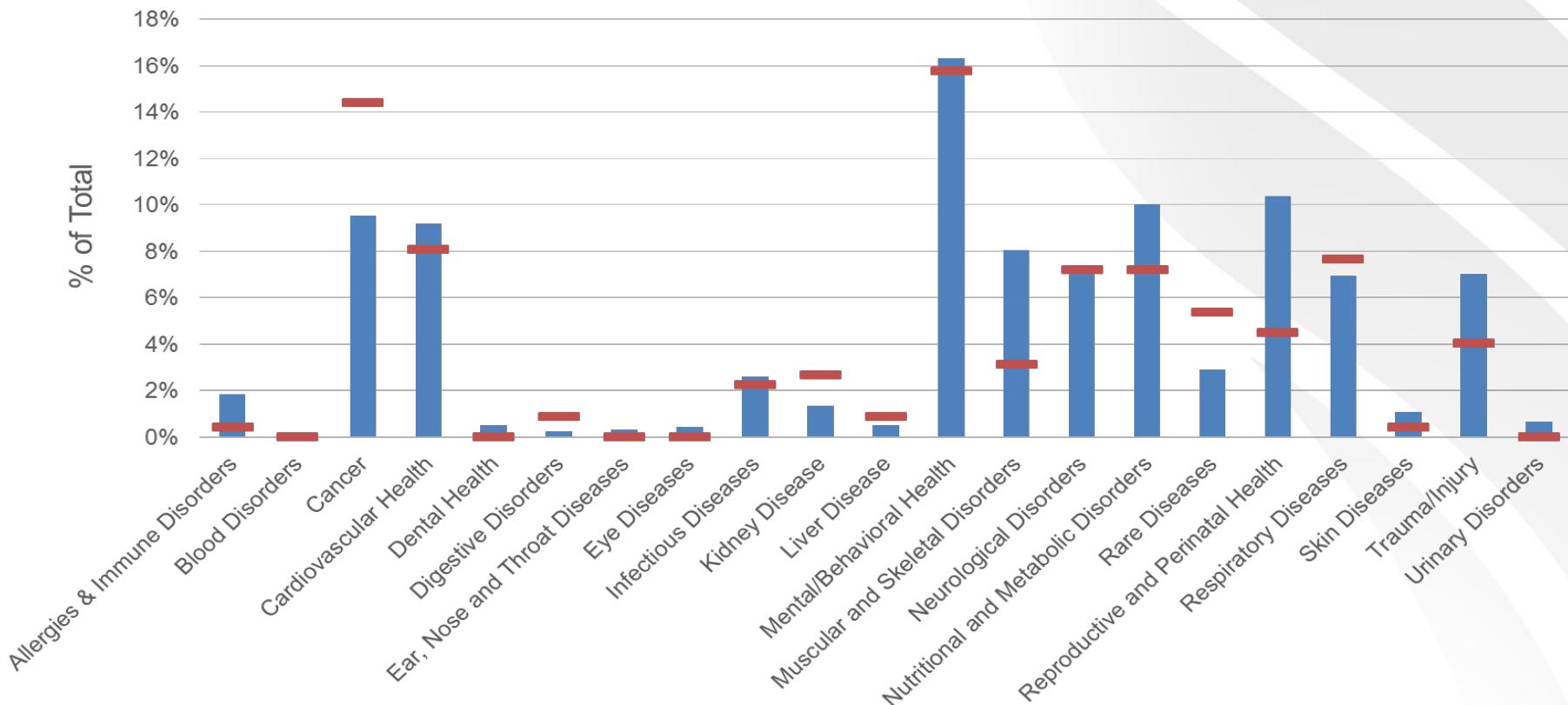
■ Prevention   ■ Diagnosis   ■ Screening   ■ Treatment   ■ Other

\*does not include projects in Improving Methods for Conducting  
Patient-Centered Outcomes Research

# Snapshot of Topics

## Submitted Topics (n=1163) v Funded Projects (n=189) by Disease/Condition\*

■ Submitted Topics ■ Funded Projects



\*For topics, this does not include topics which do not specify a disease/condition. For projects, this does not include projects which are Methods, non-disease specific, or deal with multiple chronic conditions.



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# PCORI's Research Portfolio Strategy

# Continue to Evaluate and Build Existing Research Portfolio

- Identifying clusters of research in portfolio
- Key element of our future funding strategy
- Provides opportunities to engage stakeholders to help us identify gaps within a cluster

# Continue Large Pragmatic Studies Initiative

- Funding announcements stipulate:
  - Research topics prioritized by you, our Advisory Panels
  - H:H comparisons in large, representative study populations
- We anticipate 5 funding cycles, up to \$450M total commitment, and ~45 studies under way by late 2016
  - The first awards will be announced in February 2015

# Large Pragmatic Studies

Cycle	# of LOIs Received	# of LOIs Accepted	# of Applications Received	Awards to be Announced
Spring 2014	231	35	35	February 2015
Fall 2014	168	24	16	Late April 2015
Winter 2015	132	TBD	TBD	July/August 2015

# AP Prioritized Topics Included in Large Pragmatic Studies PFAs

## APDTO

- Biologics for Treatment of Inflammatory Bowel Disease
- Identifying lung cancer in people with lung nodules
- Treatment options for opioid substance abuse
- Treatment options for multiple sclerosis
- Proton beam therapy for breast, lung, and prostate cancer
- Treatment options for autism
- Bipolar Disorder
- Ductal Carcinoma
- Chronic Kidney Disease
- Migraine Headache
- Multiple Sclerosis
- Osteoarthritis

## AD

- Interventions to Promote Tobacco Cessation Among Vulnerable Populations
- Integration of Mental and Behavioral Health Services into Primary Care Settings
- Heart attacks among racial and ethnic minorities

## IHS

- Multicomponent Interventions and Medication Adherence in Chronically Ill Patients”
- Patient and Caregiver Engagement in Chronic Mental Illness”
- Effect of Insurance Features
- Accountable care organizations
- Mental health and primary care co-location
- Effect of insurance features

# Targeted PFA Initiatives

- “Target” a single topic
- Set-aside funding (e.g. \$10-\$50M)
- Requires broad stakeholder support
- Useful for topics of urgent national concern (e.g., Hepatitis C)

# Targeted Funding Announcement Topics Approved by the Board

Targeted Funding Announcement Topics	Funding Total	Status
Clinical Trial of a Multifactorial Fall Injury Prevention Strategy in Older Persons (Administered by NIA)	\$30M	Awarded
Clinical Interventions to Reduce Hypertension Disparities (Administered through NHBRI)	\$25M	Award in 2015
Treatment Options for African Americans and Hispanics/Latinos with Uncontrolled Asthma	\$24M	Awarded
Obesity Treatment Options Set in Primary Care for Underserved Populations	\$20M	Awarded
PCOR Treatment Options in Uterine Fibroids (Administered by AHRQ)	\$20M	Awarded
Effectiveness of Transitional Care	\$15M	Awarded
Optimal Maintenance Aspirin Dose for Patients with Coronary Artery Disease (PCORnet demonstration trial)	\$10M	Award in FY 2015
<b>Targeted PFA Total:</b>	<b>\$144M</b>	

# Proposed Percent Distribution Annual Funding Commitment

Commitment Period (\$ in Millions, unless noted)				
Fiscal Period	Research	Broad	Pragmatic	Targeted*
FY 2012-2013	\$331			
FY 2014	\$300	69%	0%	31%
FY 2015	\$475	25%	55%	20%
FY 2016	\$400			
FY 2017	\$300			
FY 2018	\$300			
FY 2019	\$200	~30%	~20%	~50%
Cumulative Total (%)	~\$2.3 B (100%)	~\$917 (40%)	~\$655 (28%)	~\$734 (32%)

\*This will be a blend of approaches to targeted funding; some may be single targeted topics and some may be Priority Topics (clusters of studies).

# Encourage the Use of PCORnet, When Appropriate

- PCORnet to be “research ready” late 2015
- Expecting applicants to propose using PCORnet
- Research in PCORnet will:
  - Be conducted in ‘real world’ settings
  - Take less time
  - Be less expensive

# Funding Vision

- Continue but taper broad research program
  - Continue to evaluate and build upon existing portfolio to identify and strategically manage clusters of research projects
- Continue to target key topics
- Continue Large Pragmatic Clinical Studies program
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## Final Comment

**This funding strategy is the continuing evolution of our shift to larger, targeted, more stakeholder-based patient-centered outcomes research.**

# Questions?



# **Evaluating PCORI's Approach to Topic Generation and Prioritization**

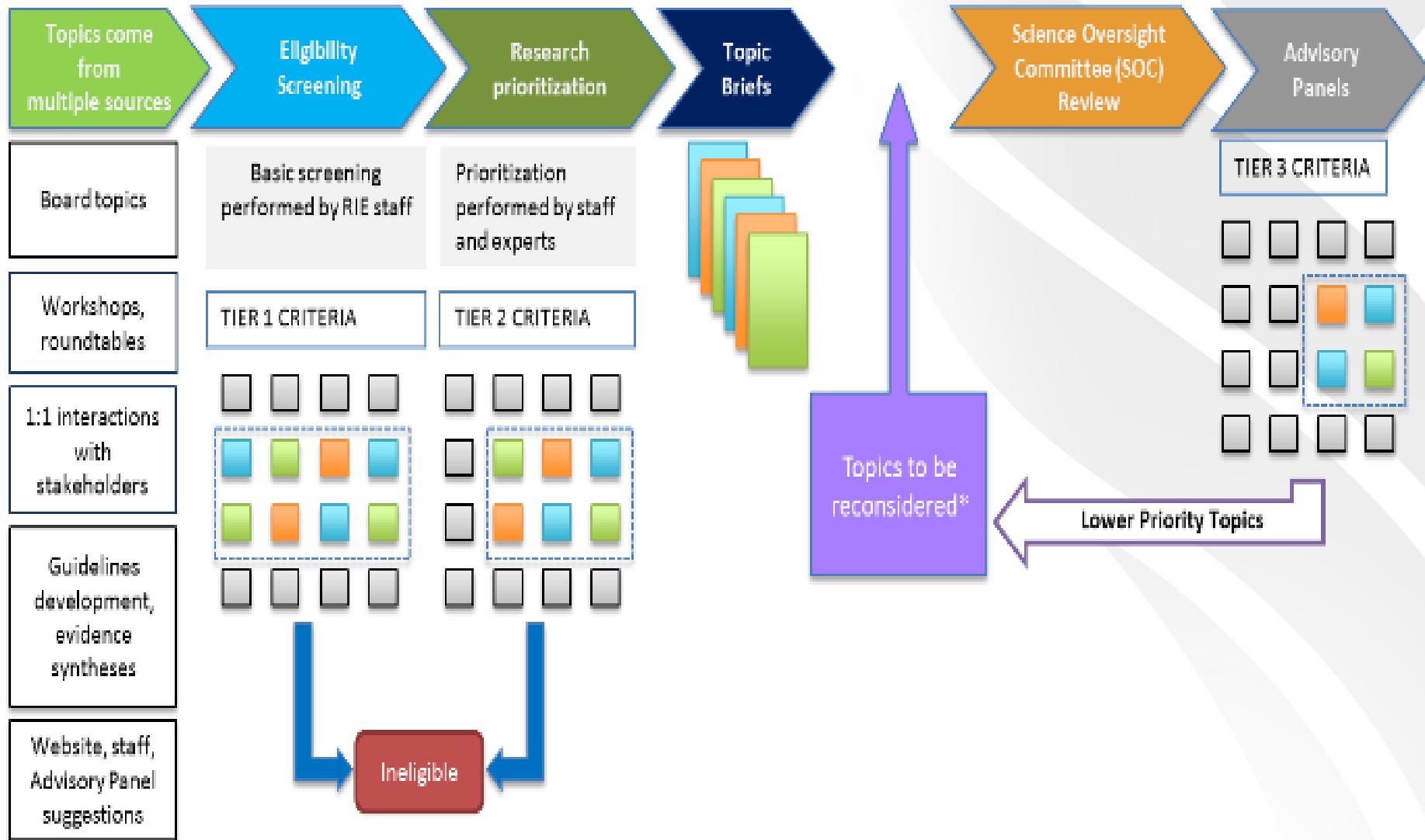
Laura Forsythe, PhD, MPH

Senior Program Officer, Research Integration and  
Evaluation Program

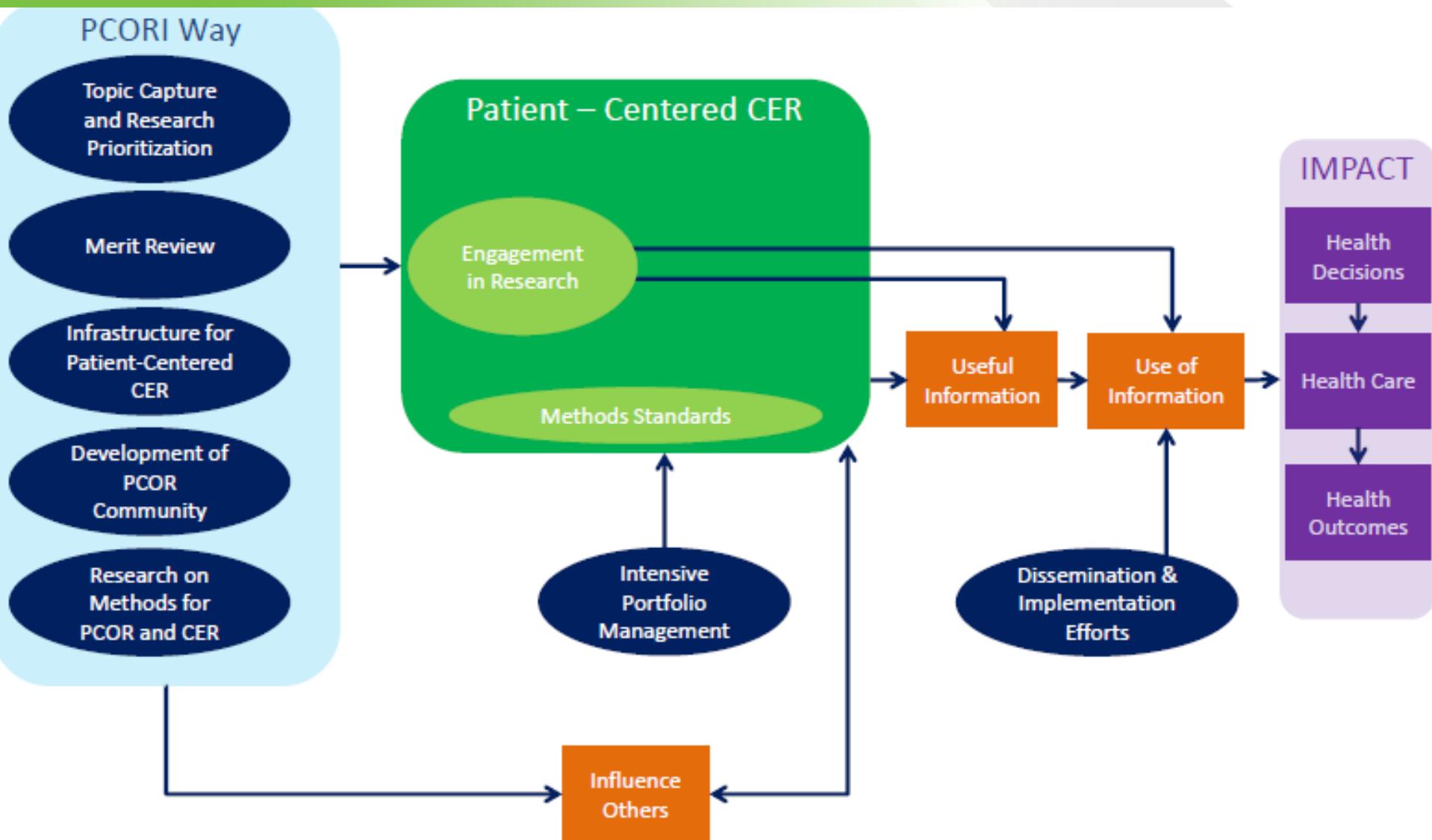
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# Objectives

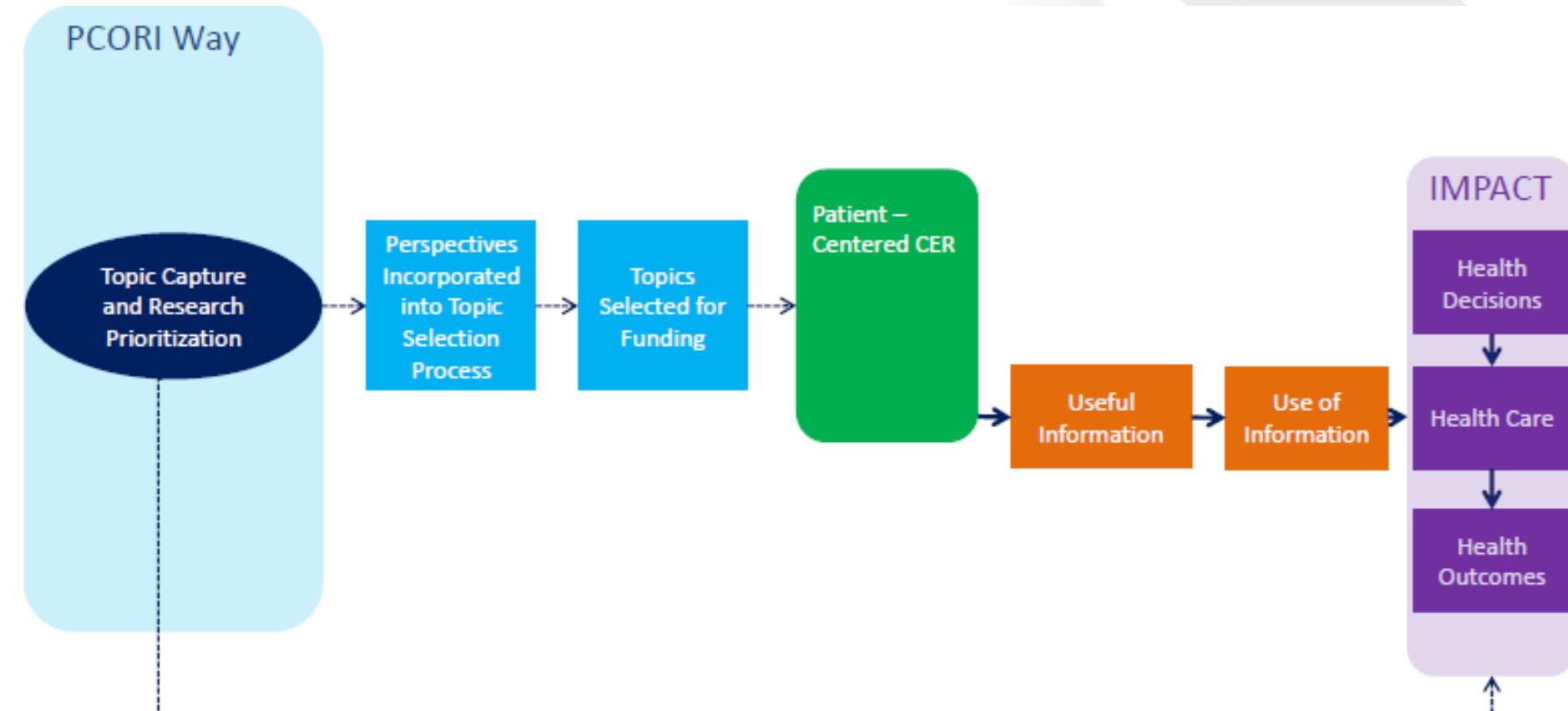
- PCORI's approach to evaluation
- Examining the TGRP process
  - Evaluation questions
  - Early learnings



# PCORI Evaluation Framework



# PCORI Evaluation Framework: TGRP



# Topic Generation and Research Prioritization

## Evaluation Questions

- What is the impact of PCORI's approach to Topic Generation, Prioritization, and Selection (inclusion of patients and other stakeholders, methods for ranking and selection) on:
  - perspectives incorporated into topic selection process,
  - the topics selected for funding, and
  - PCORI projects filling identified research gaps?

# Topic Generation and Research Prioritization

## Evaluation Questions

- What is the impact of PCORI's approach to Topic Generation, Prioritization, and Selection (inclusion of patients and other stakeholders, methods for ranking and selection) on:
  - **perspectives incorporated into topic selection process,**
  - the topics selected for funding, and
  - PCORI projects filling identified research gaps?

# Perspectives incorporated into topic selection process

- Describe perspectives captured through public topic solicitation and engagement outreach
- Track progress of topics through prioritization
- Comparison of submissions against funded portfolio
- Examine perceptions of Advisory Panelists

# Hearing from Stakeholders

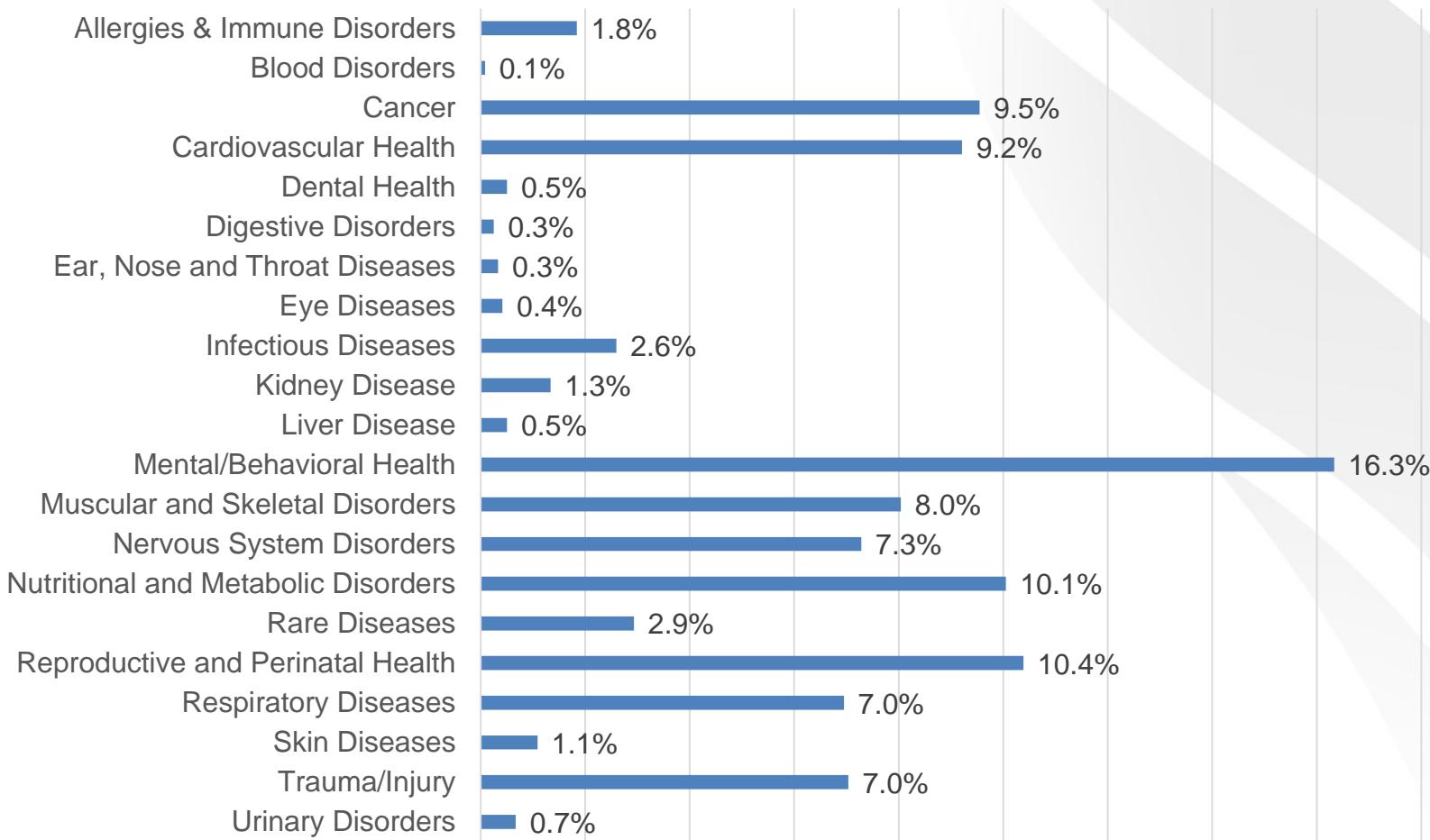
## PCORI Engagement Workshop, November 2012

- Patients don't have research questions, they have questions
- There are research questions others have identified that PCORI should address
- Provide tracking of progress of topics through prioritization

# Perspectives Captured through Topic Solicitation

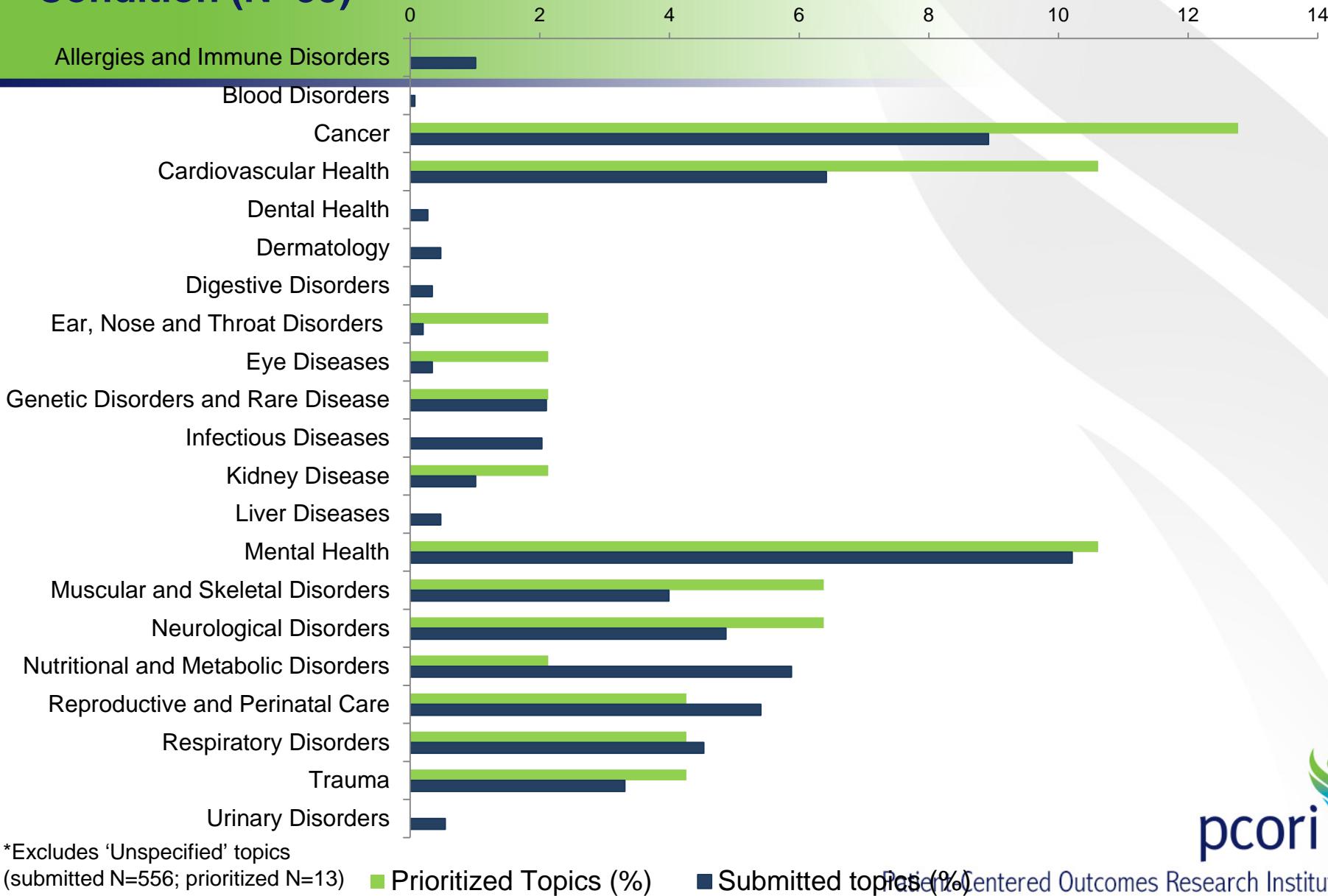
	SH1	SH2	SH3	SH4	SH5	...	IOM 100	PCORI Website Submissions
Fall Injury Prevention in Older Persons							x	33 topics
Treatment Options in Uterine Fibroids								24 topics
Hypertension		x	x		x		x	21 topics
Perinatal Care and Outcomes		x			x			47 topics
Treatment Options for Back Pain	x	x					x	36 topics

# Submitted Topics by Disease/Condition\*

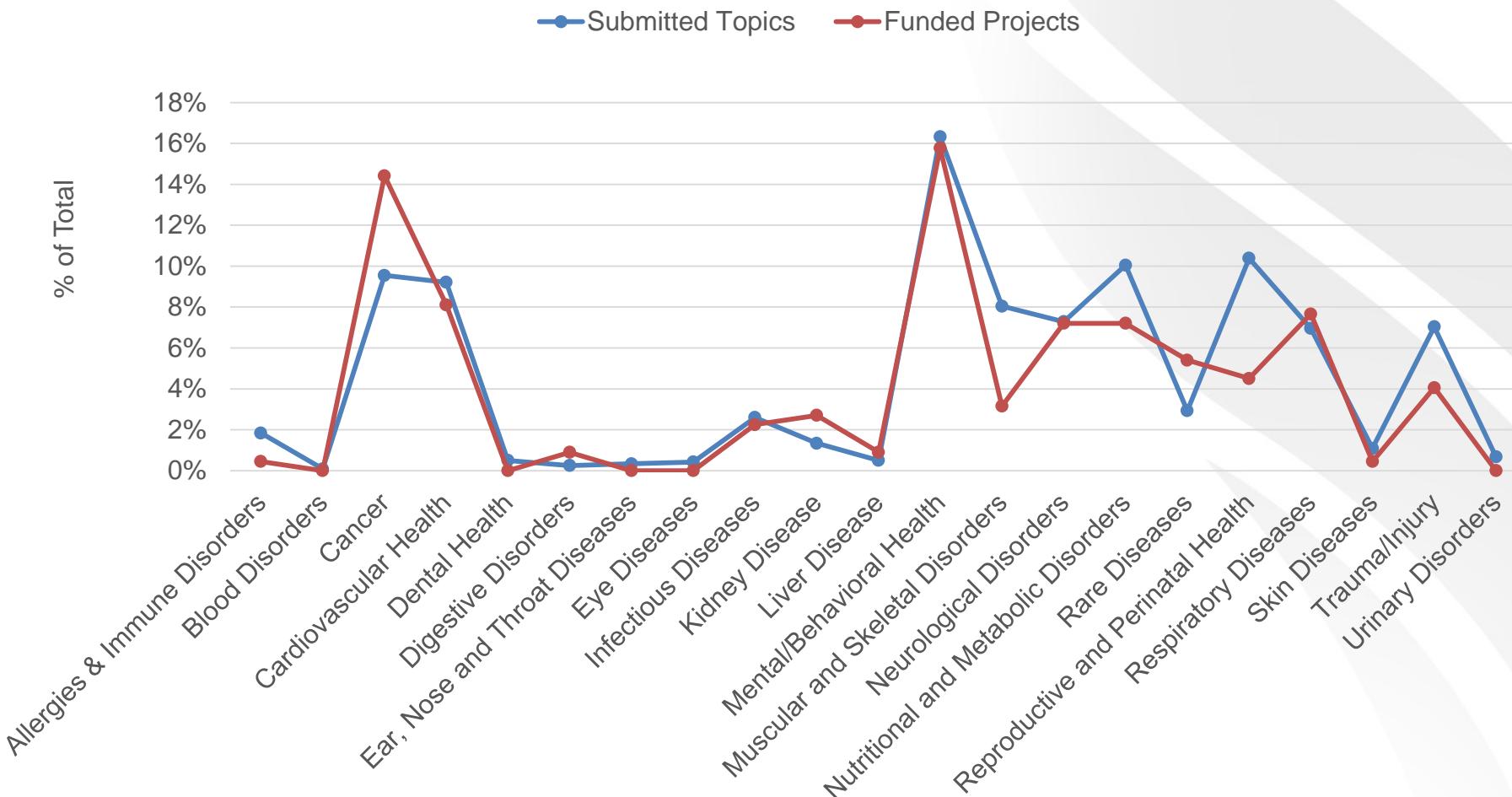


\*Excludes topics where the disease/condition is "Unspecified"

# Spring 2013 Submitted Topics (N=923) and Prioritized Topics by Condition (N=33)\*

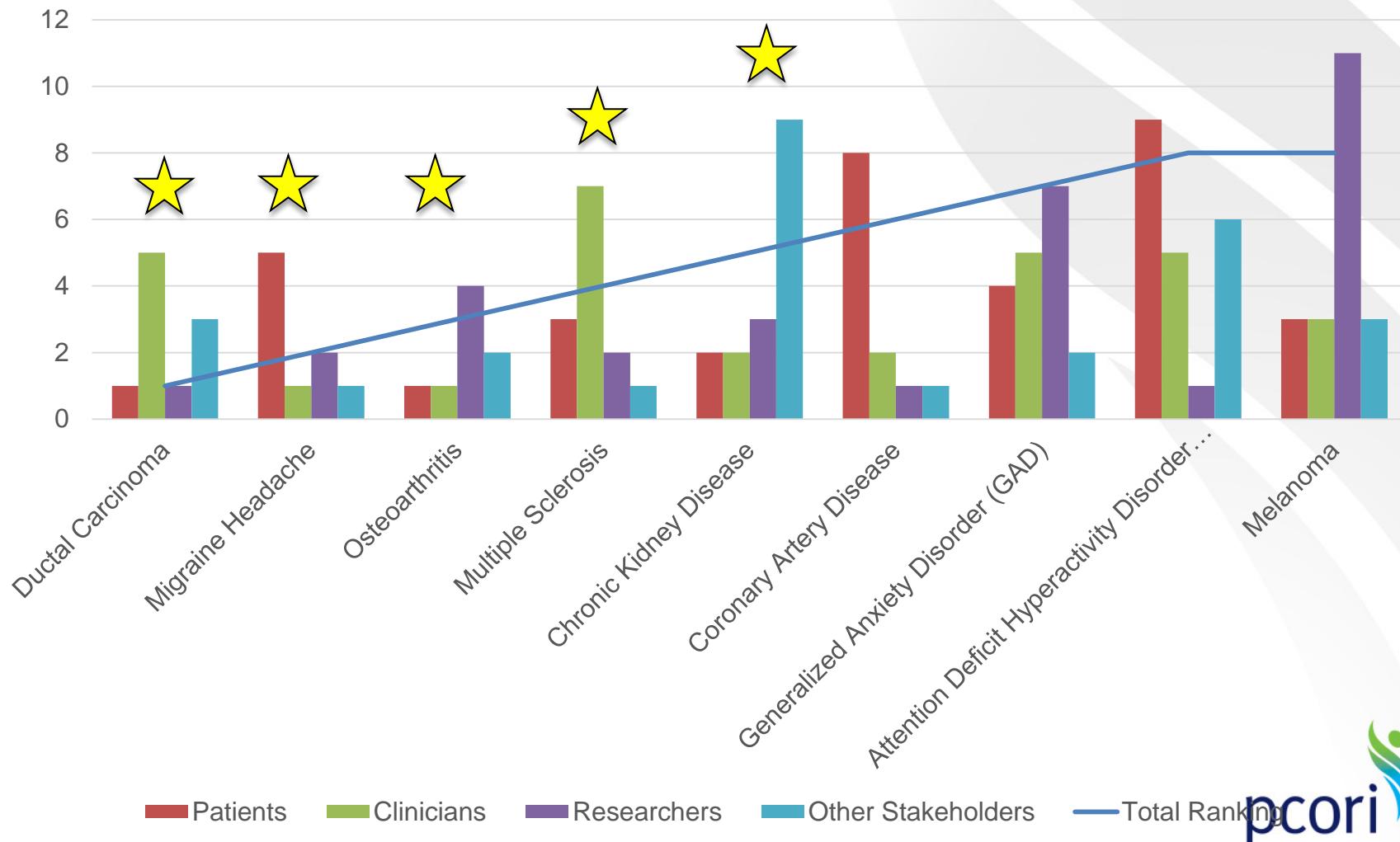


## Submitted Topics (n=1163) v Funded Projects (n=189) by Disease/Condition\*

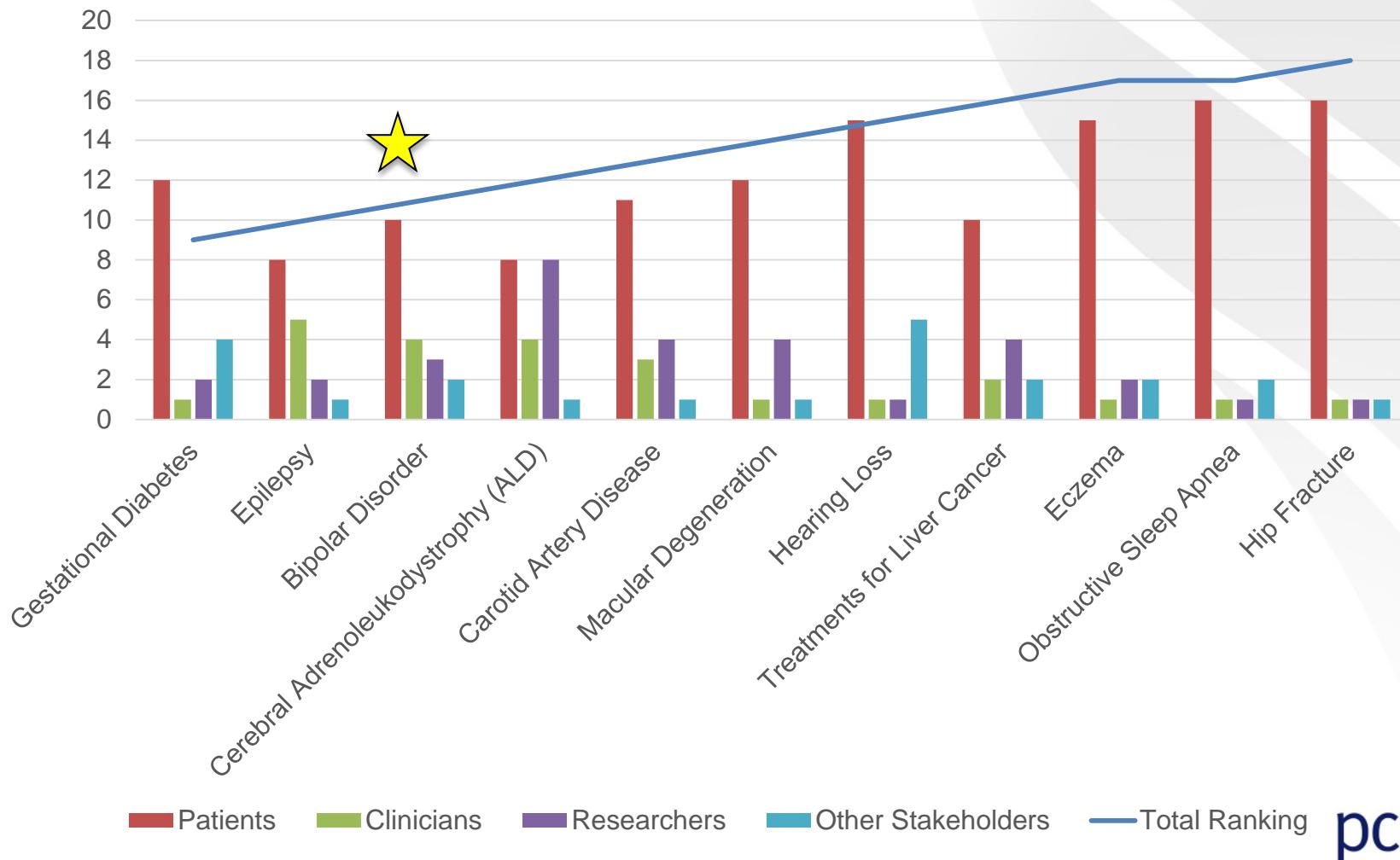


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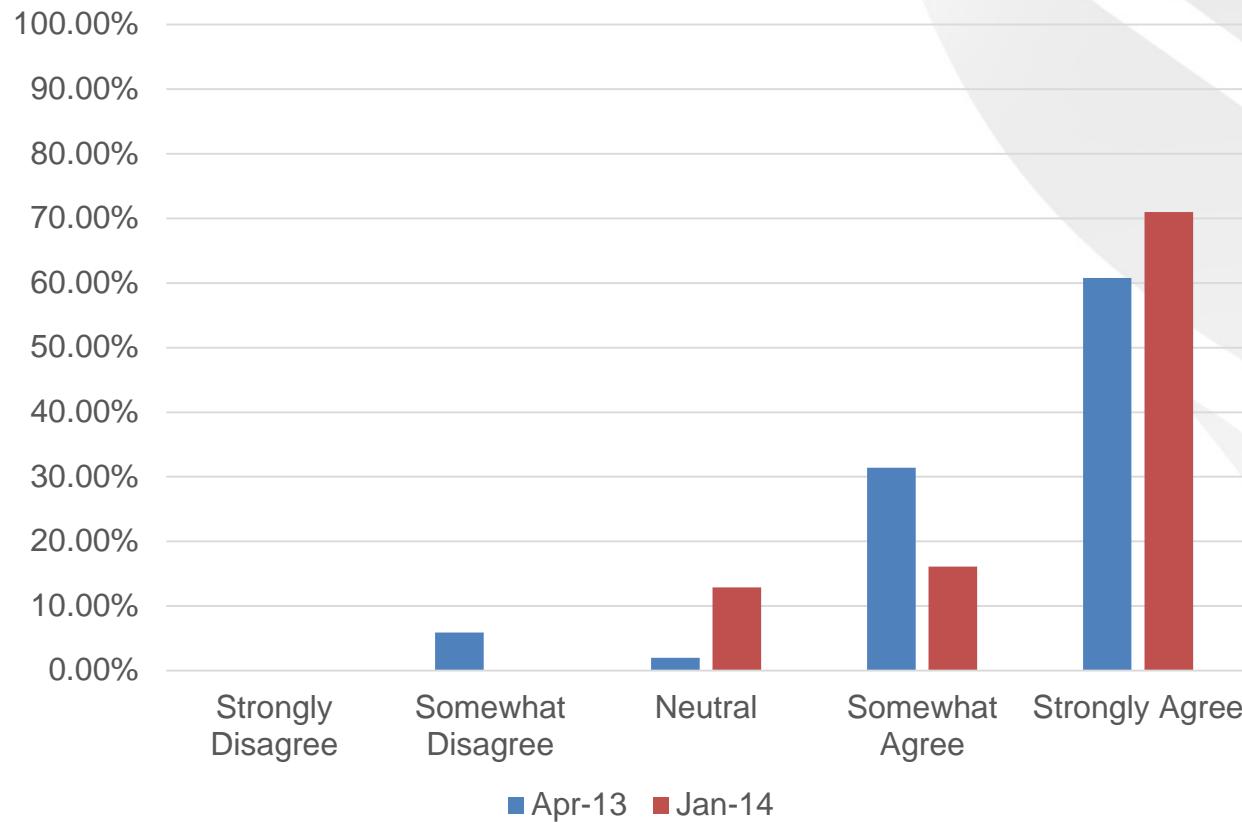
# April 2013 Prioritization by Stakeholder APDTO Panel (1 of 2)



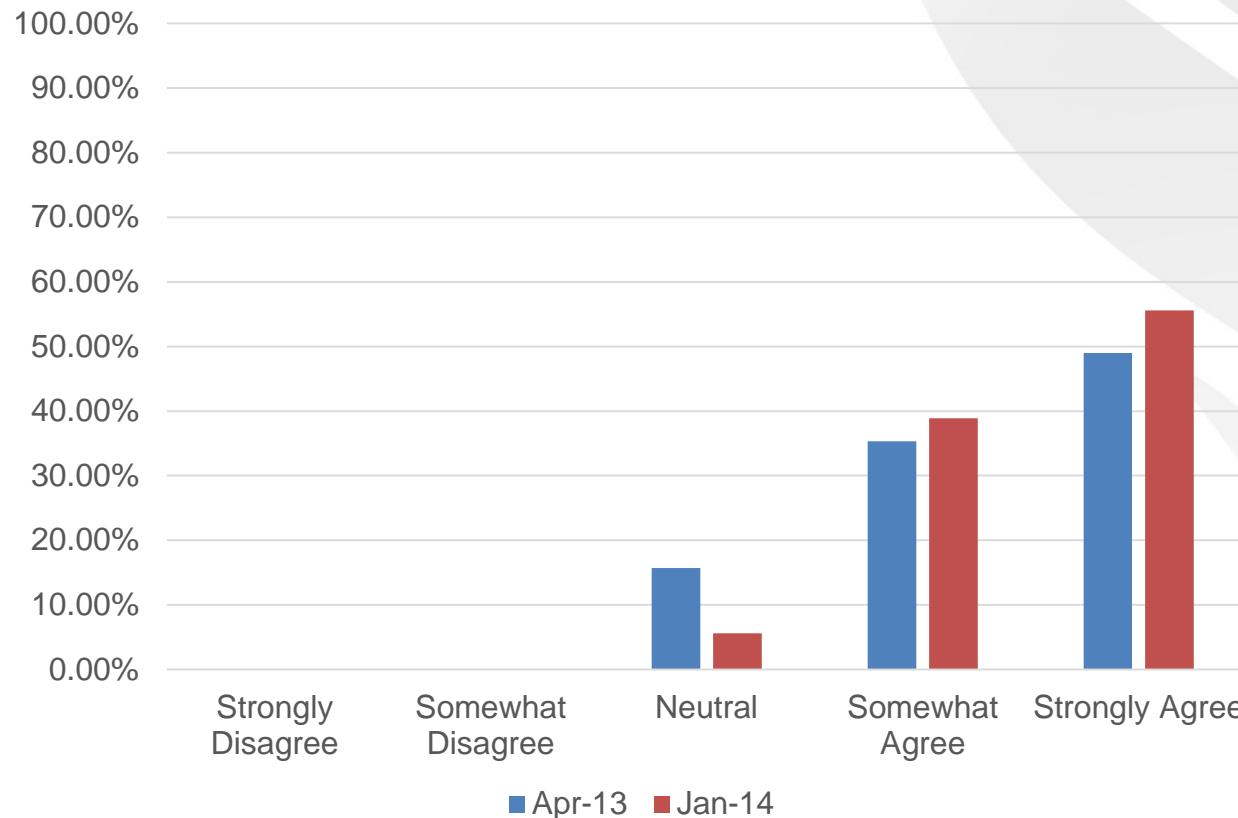
# April 2013 Prioritization by Stakeholder APDTO Panel (2 of 2)



# Poll: The Advisory Panel meeting gave me the opportunity to provide input on PCORI's research topics



# Poll: PCORI's method for research topic prioritization will help PCORI fund research that can inform health care decisions by patients



# Topic Generation and Research Prioritization

## Evaluation Questions

- What is the impact of PCORI's approach to Topic Generation, Prioritization, and Selection (inclusion of patients and other stakeholders, methods for ranking and selection) on:
  - perspectives incorporated into topic selection process,
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# Topics selected and Gaps filled

- Portfolio comparison- PCORI vs. others
- Stakeholder rating of topic relevance
- Usefulness to end users

# Summary and Discussion

- PCORI is evaluating topic generation and research prioritization along with other PCORI work.
- We are looking at the:
  - types of stakeholders that submit topics
  - contribution to prioritization by stakeholder type
  - comparison of submitted topics to PCORI-funded topics.
- What other information should we collect to evaluate the impact of our topic generation and research prioritization process?

# Questions?

# Individual Panel Meeting Rooms

- Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options (Crystal Ballroom Salon C)
- Advisory Panel on Addressing Disparities (Lincoln Hall – Lower Level)
- Advisory Panel on Patient Engagement (Crystal Ballroom Salon B)
- Advisory Panel on Rare Disease (Pentagon 1 & 2)