



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.	General Session: <ul style="list-style-type: none">• PCORI's Funding Strategy – B. Luce• Evaluating PCORI's Approach to Topic Generation, Prioritization, and Selection – L. Forsythe
9:15 a.m.	Break
9:30 a.m.	Individual Panel Meetings: <ul style="list-style-type: none">• 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)

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">• PCORI's Funding Strategy – B. Luce• Evaluating PCORI's Approach to Topic Generation, Prioritization, and Selection – L. Forsythe
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"> • Advisory Panel on Improving Healthcare Systems (Crystal Ballroom Salon C) • Advisory Panel on Patient Engagement (Crystal Ballroom Salon B) • Advisory Panel on Clinical Trials (Pentagon 1 & 2)
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"> • Advisory Panel on Improving Healthcare Systems (Crystal Ballroom Salon C)

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?

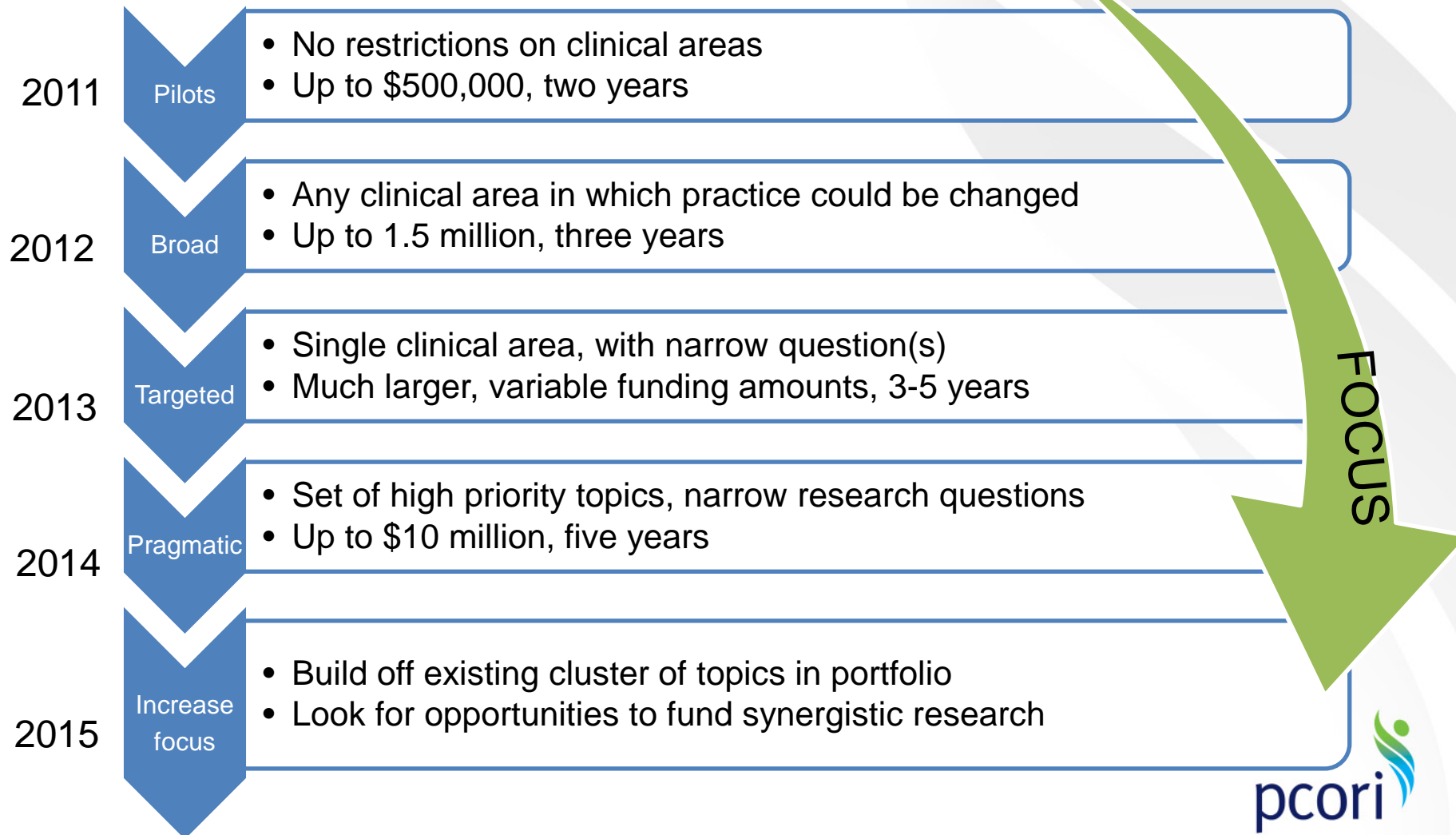
Specific Issues To Improve Funding Process (2)

- 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

Specific Issues To Improve Funding Process (3)

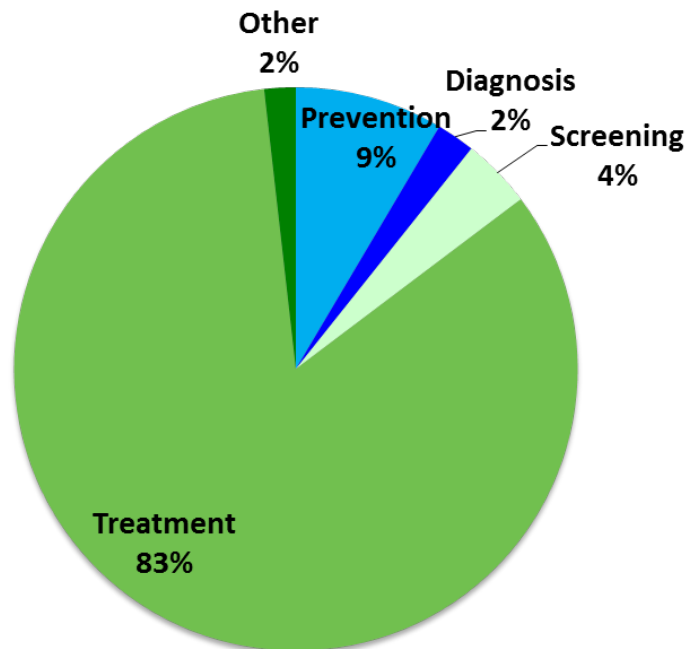
- Growing interest in “cross-cutting” health CER, e.g. syndromes, symptoms (pain, anxiety)
 - Generally
 - Rare diseases (RDAP)
- Improving HealthCare Systems CER
 - Active Collaborating with AHRQ
 - Evaluating appropriate comparator designs (e.g. “usual care”)
- Funding CER in PCORnet
 - Proof of concept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

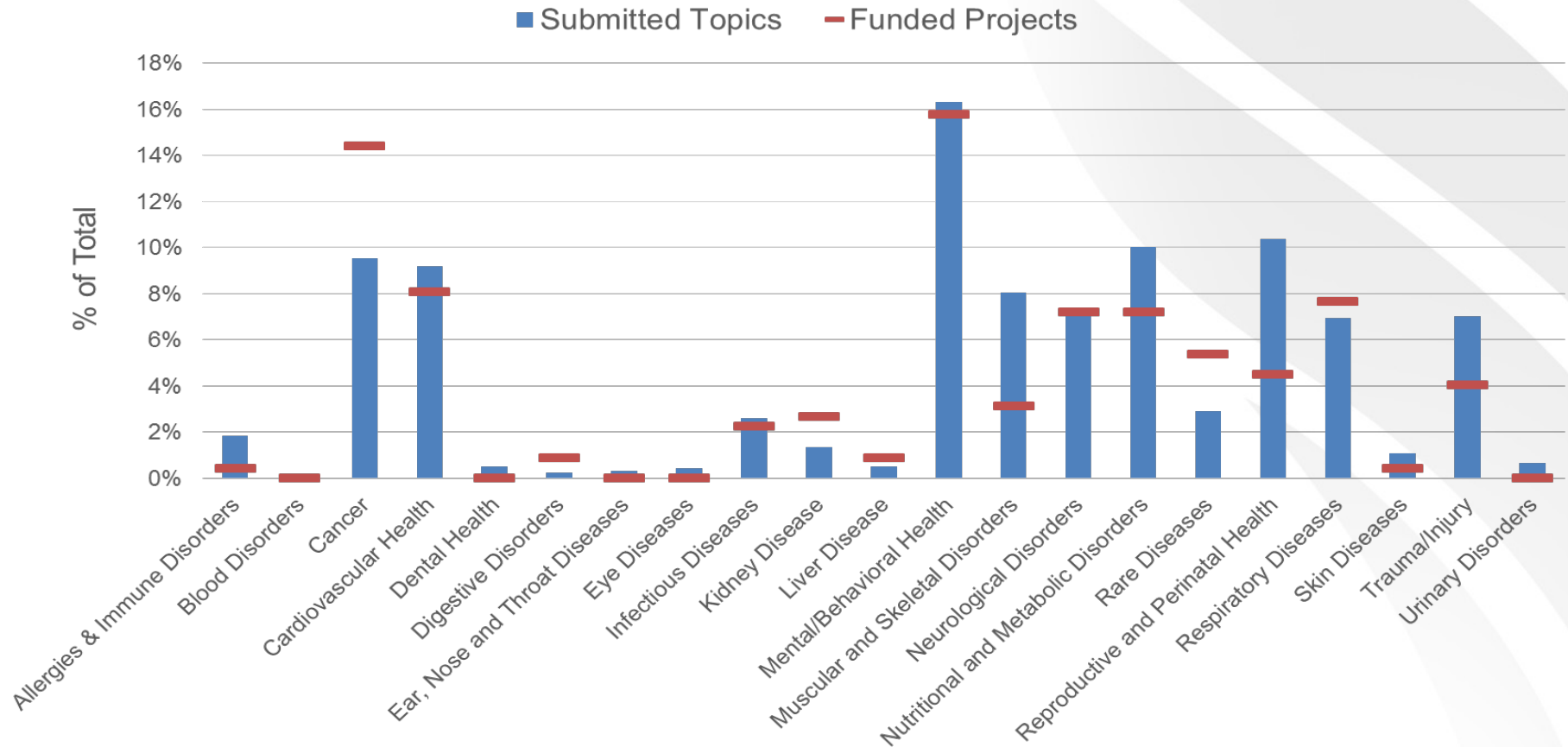
*does not include projects in Improving Methods for Conducting
Patient-Centered Outcomes Research



Patient-Centered Outcomes Research Institute

Snapshot of Topics

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



*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.

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 NHBLI)	\$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
Targeted PFA Total:	\$144M	

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).



pcori

Patient-Centered Outcomes Research Institute

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
- Prepare and operationalize PCORnet for CER research

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

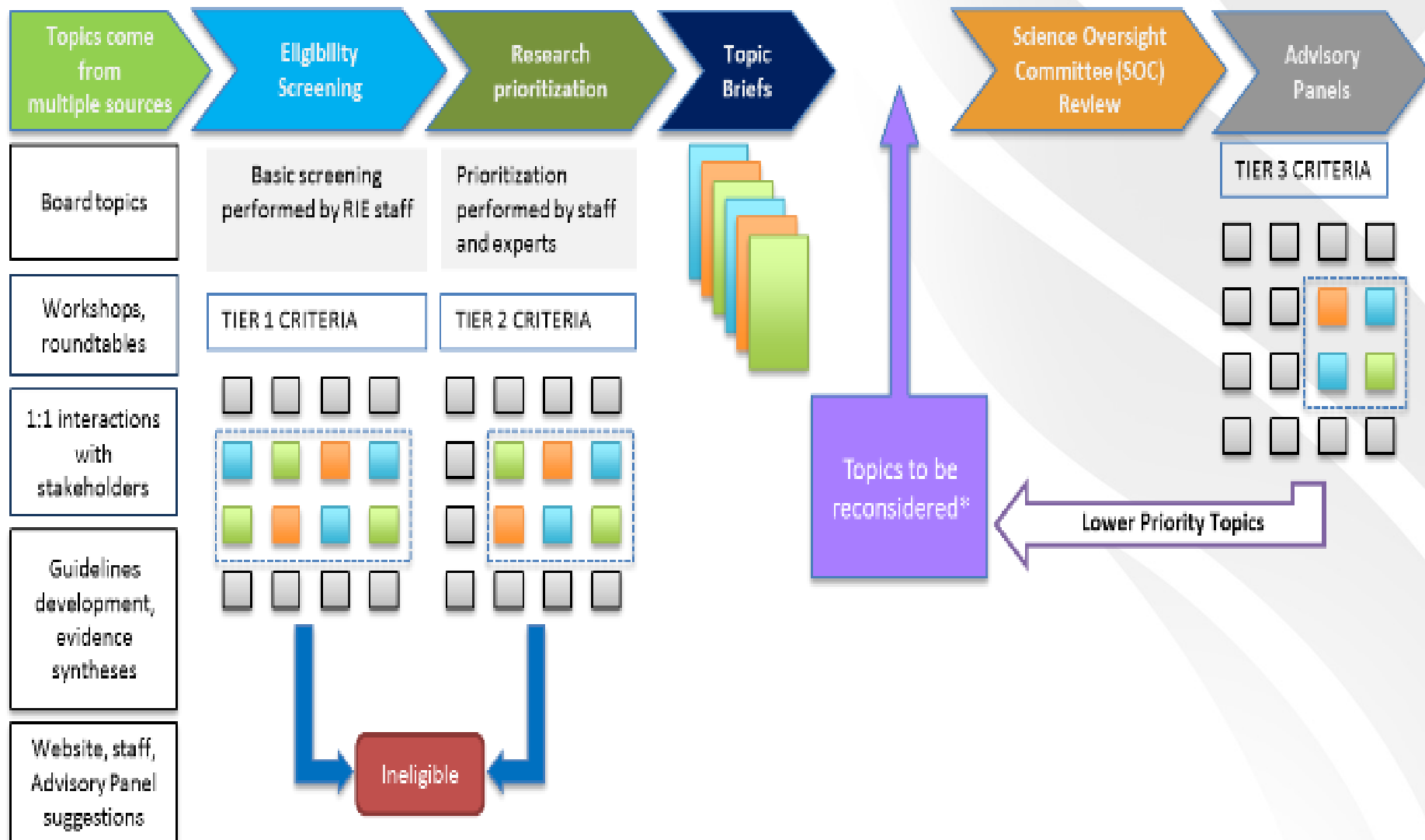
Laura Forsythe, PhD, MPH

Senior Program Officer, Research Integration and
Evaluation Program

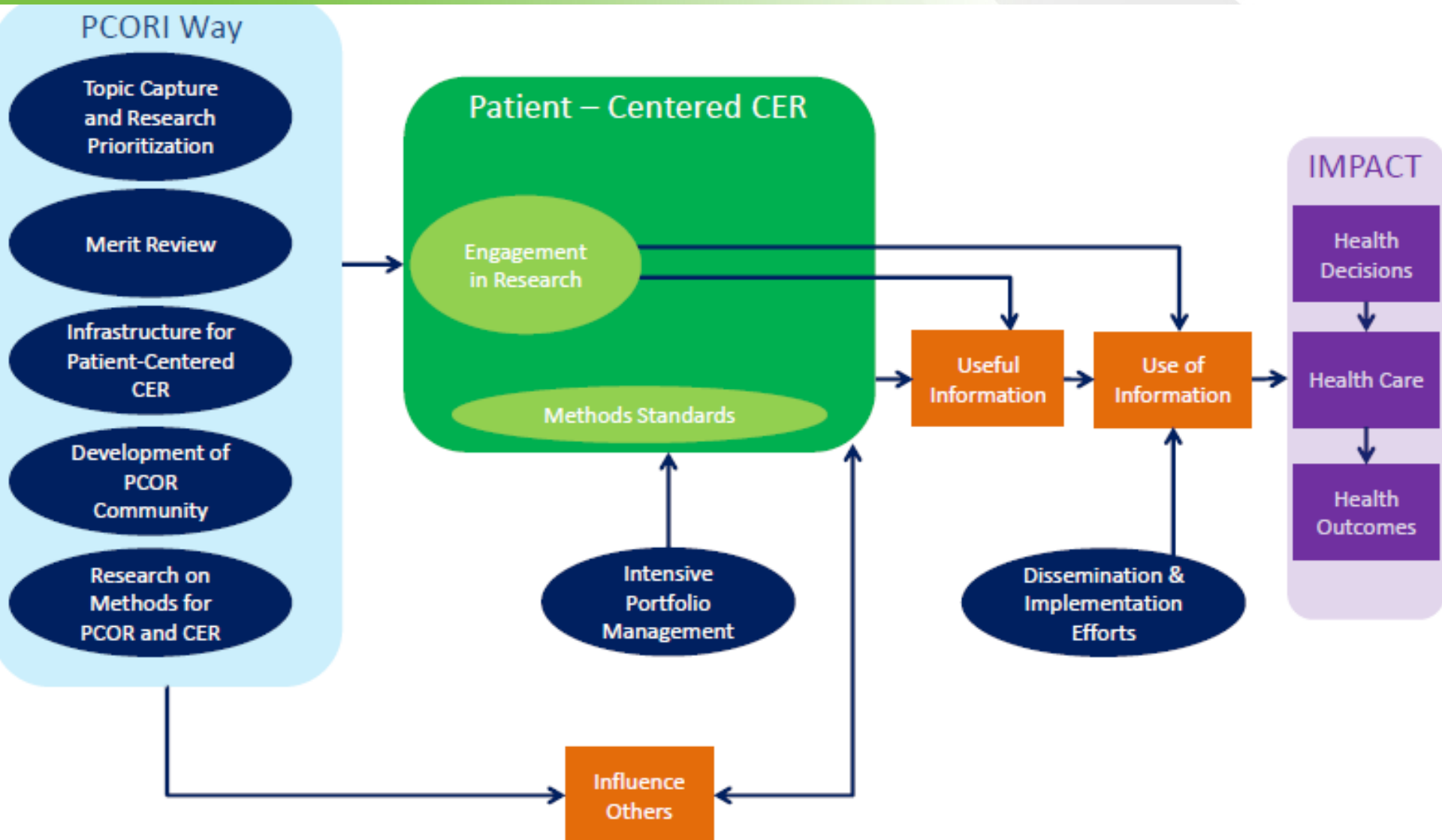
Patient-Centered Outcomes Research Institute

Objectives

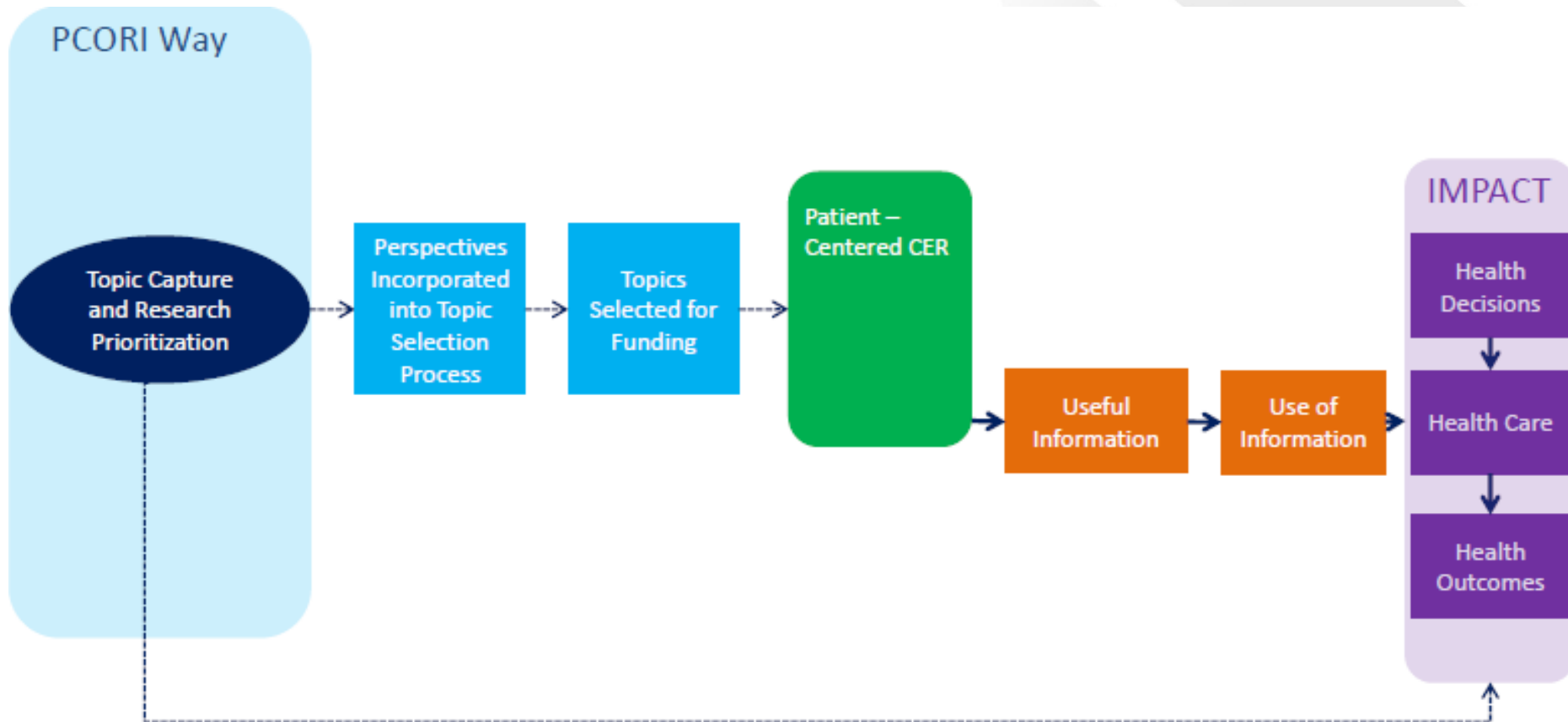
- PCORI's approach to evaluation
- Examining the TGRP process
 - Evaluation questions
 - Early learnings



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

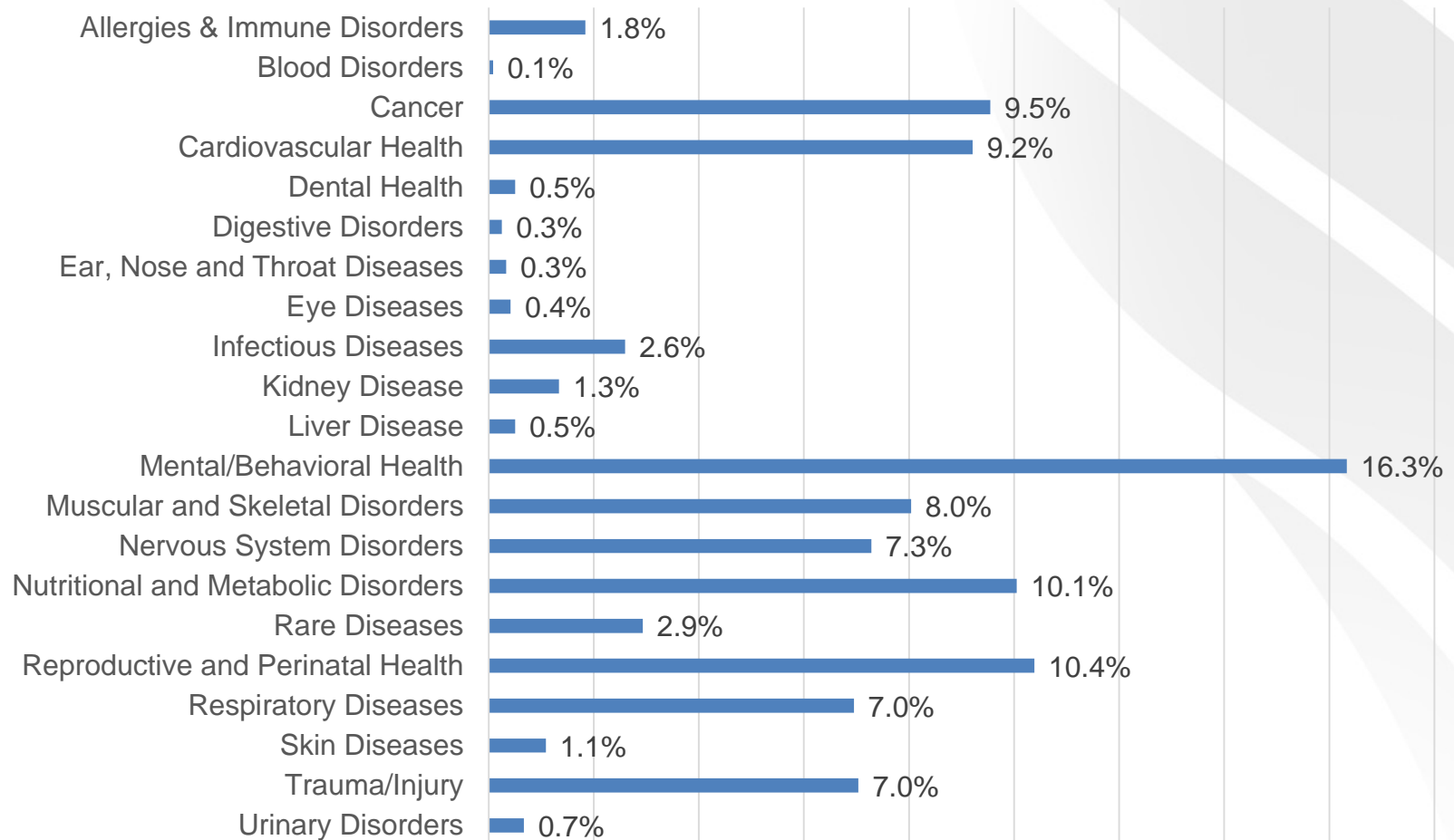
PCORI Engagement Workshop, November 2012

- 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

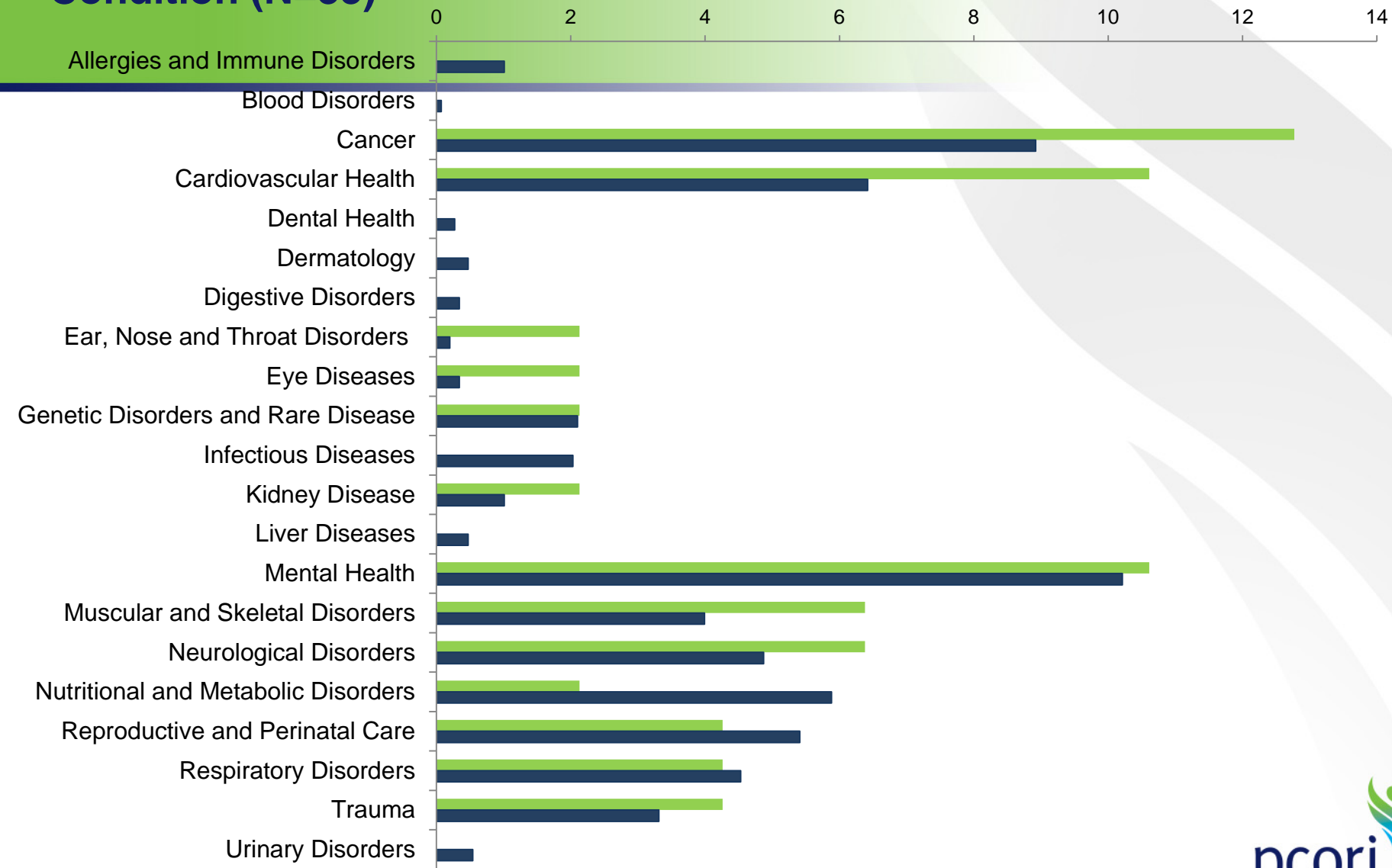
	SH1	SH2	SH3	SH4	SH5	...	IOM 100	PCORI Website Submissions
Fall Injury Prevention in Older Persons							x	33 topics
Treatment Options in Uterine Fibroids								24 topics
Hypertension		x	x		x		x	21 topics
Perinatal Care and Outcomes		x			x			47 topics
Treatment Options for Back Pain	x	x					x	36 topics

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)*



*Excludes 'Unspecified' topics

(submitted N=556; prioritized N=13)

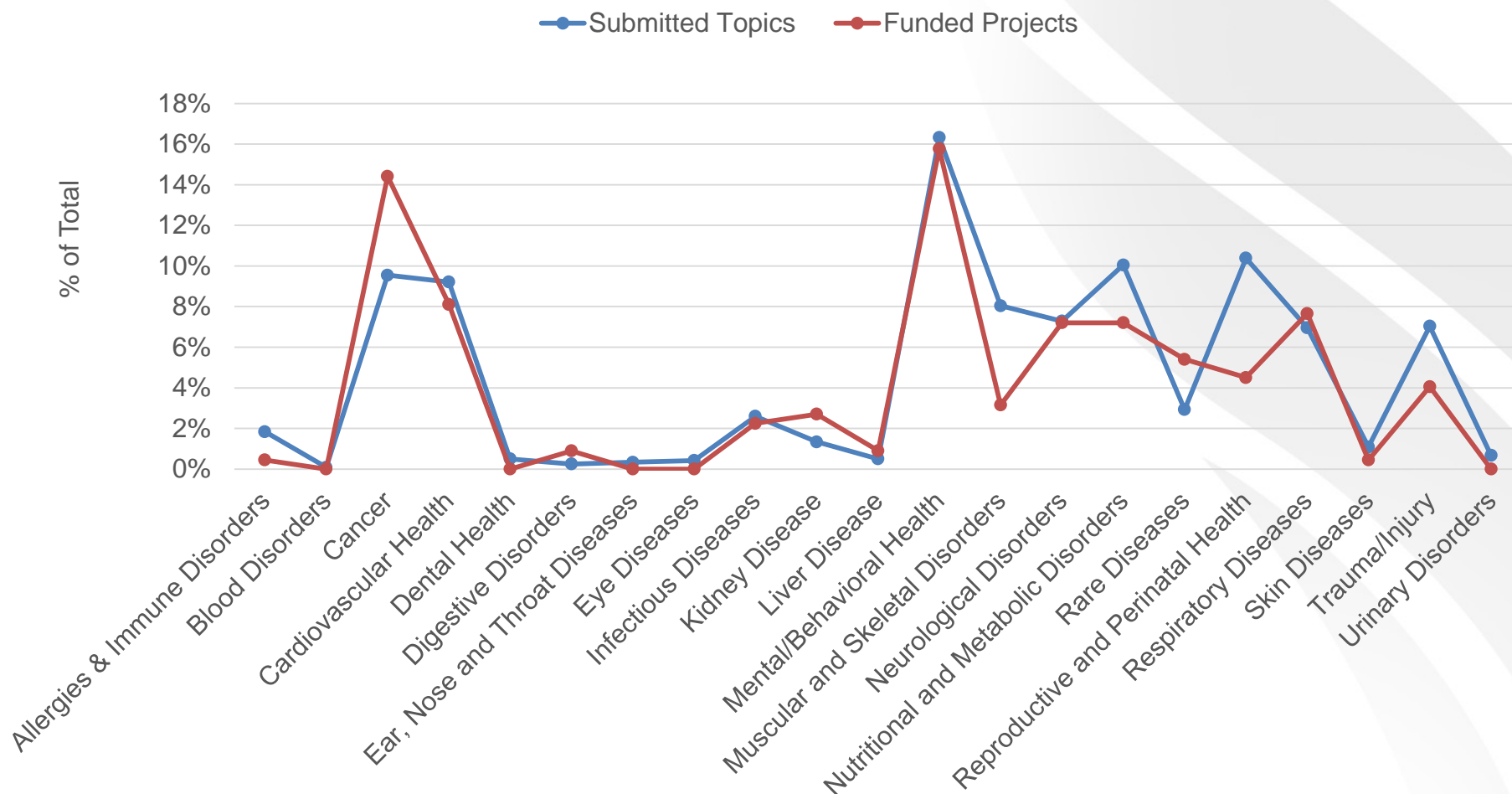
■ Prioritized Topics (%)

■ Submitted topics (%)

Perinatal Centered Outcomes Research Institute



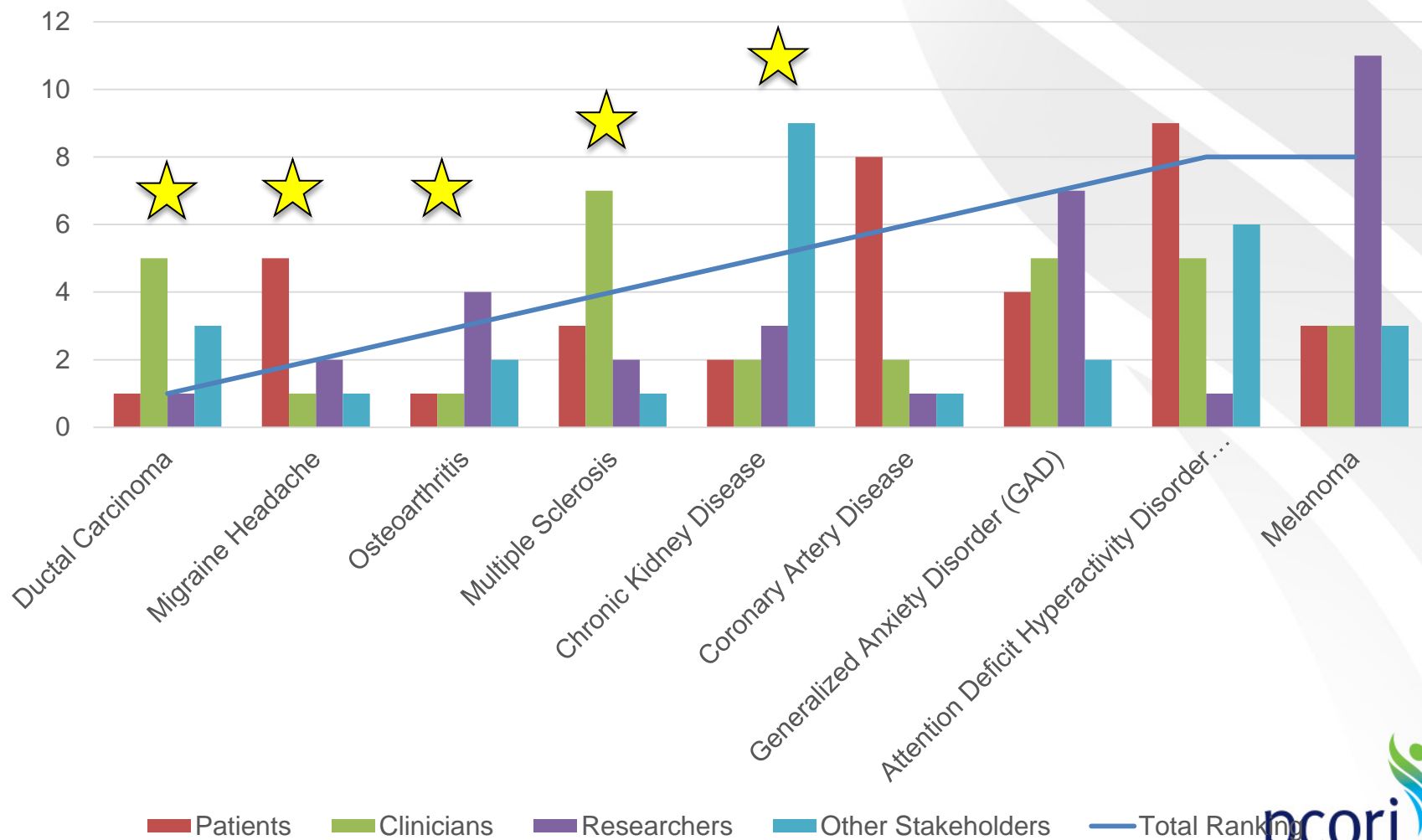
Submitted Topics (n=1163) v Funded Projects (n=189) by Disease/Condition*



*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.

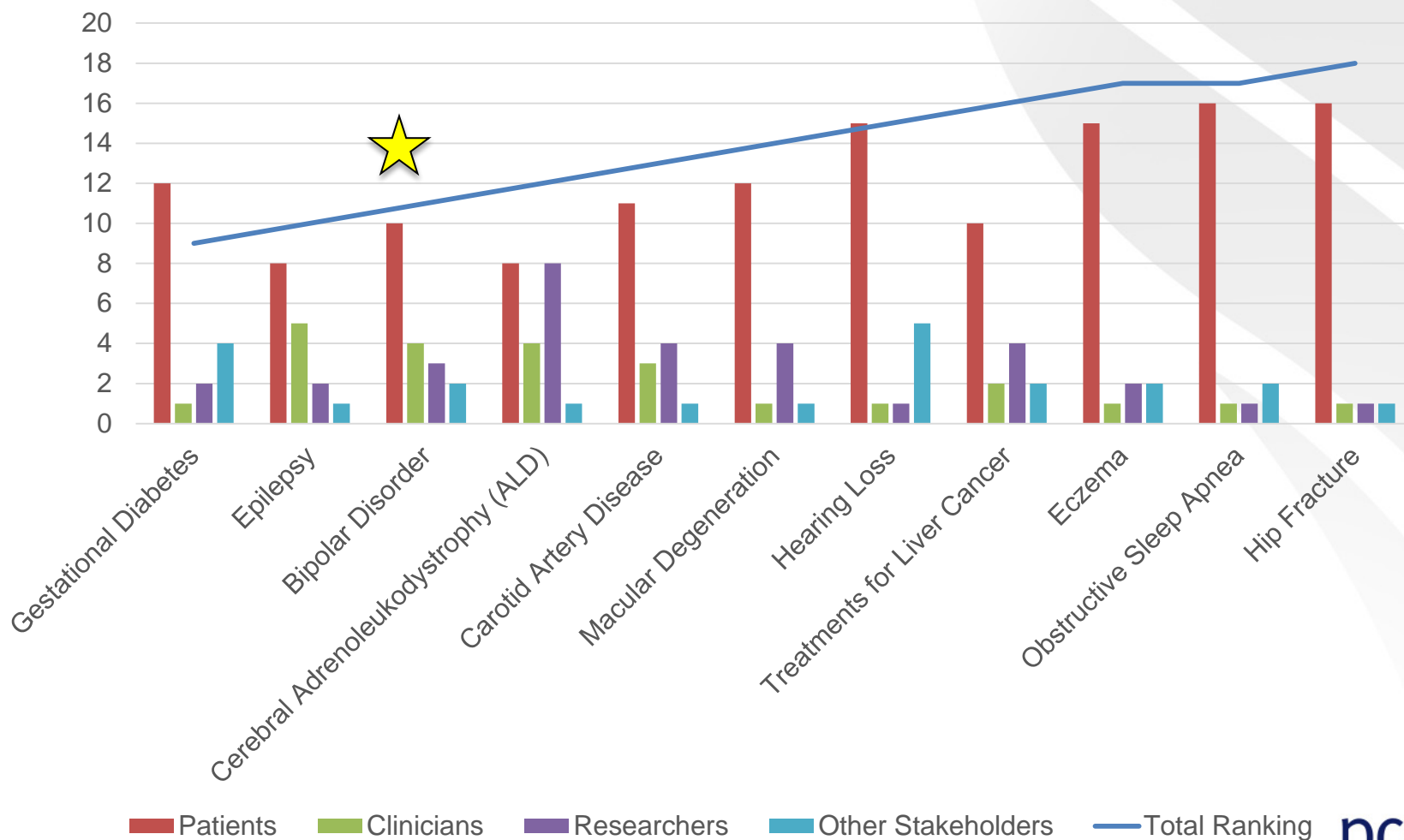
April 2013 Prioritization by Stakeholder

APDTO Panel (1 of 2)

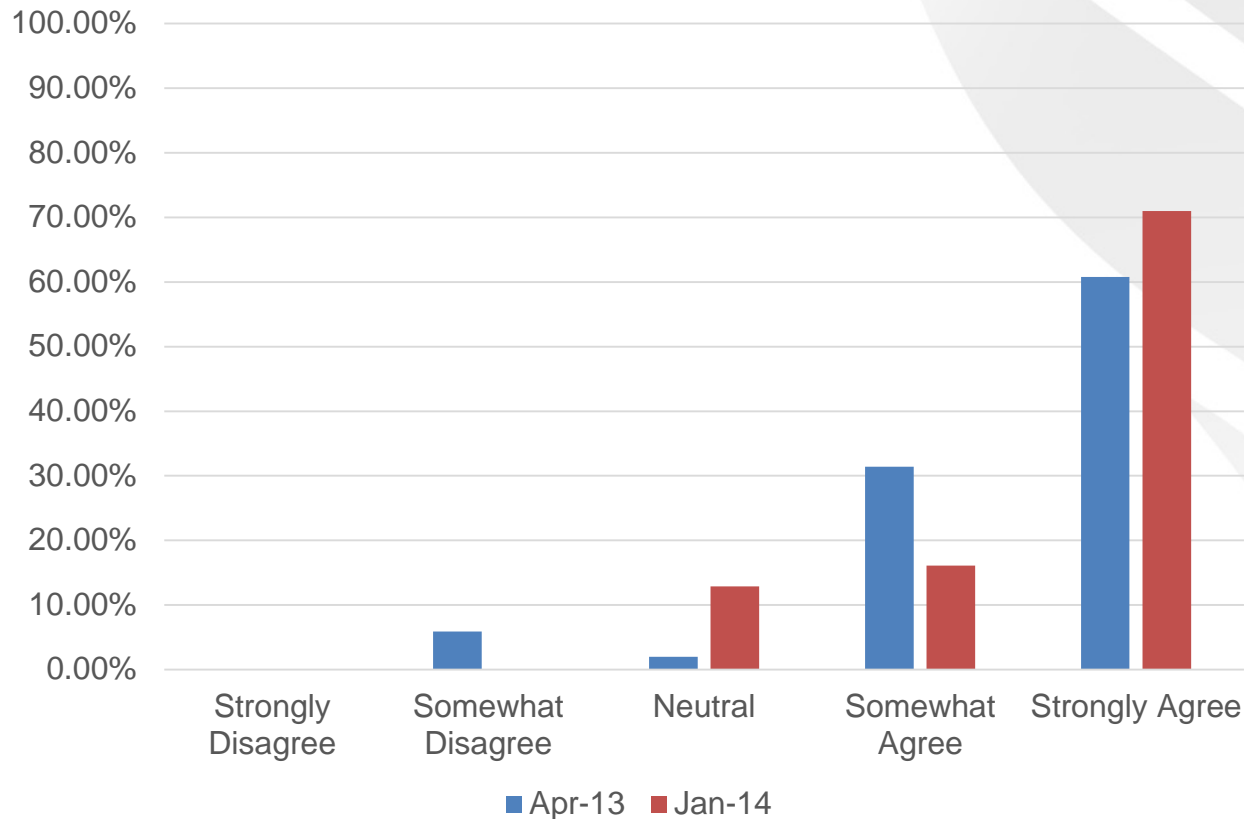


April 2013 Prioritization by Stakeholder

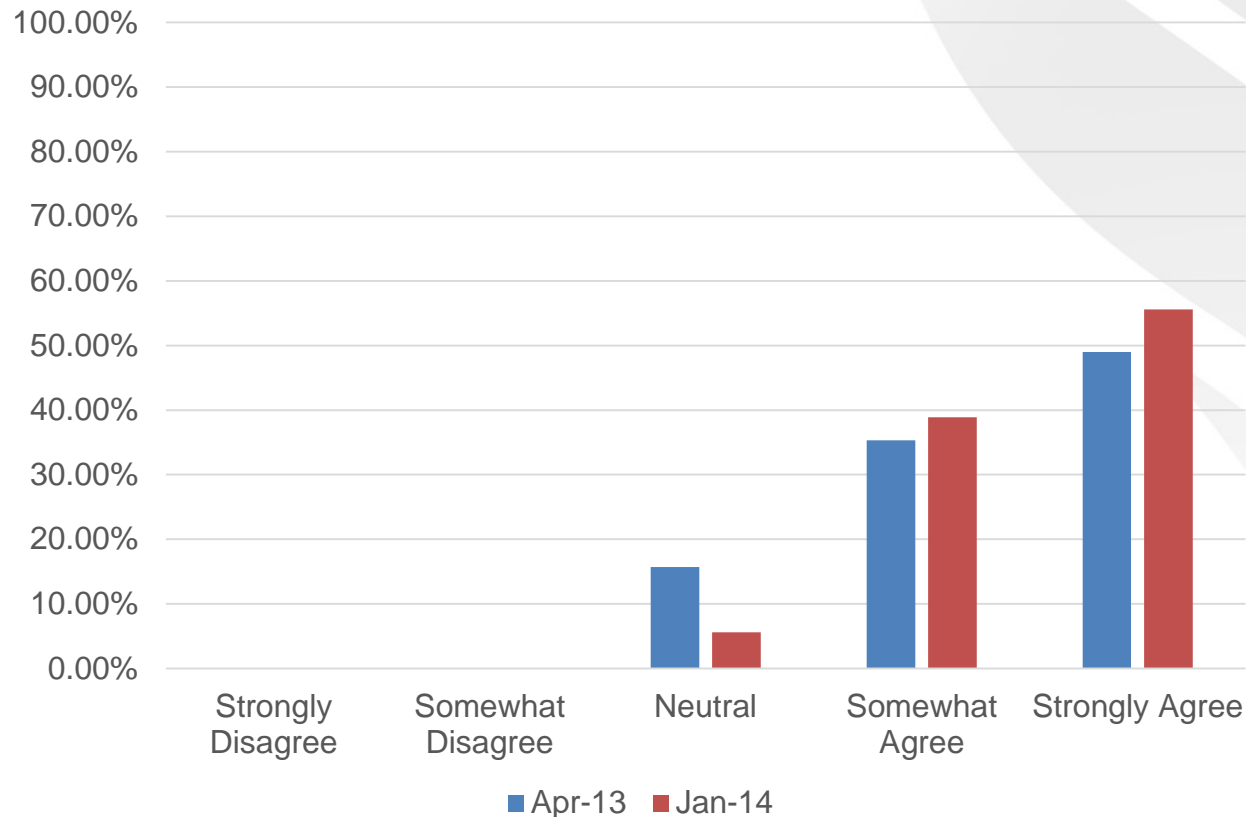
APDTO Panel (2 of 2)



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,
 - the topics selected for funding, and
 - PCORI projects filling identified research gaps?

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)