

# Board of Governors Meeting via Teleconference/Webinar

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August 16, 2016  
12:00-1:30 p.m. ET



PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

# Welcome and Introductions

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**Grayson Norquist, MD, MSPH**

*Chairperson, Board of Governors*

**Joe Selby, MD, MPH**

*Executive Director*



# Agenda

| Time          | Agenda Item                                                                                                                                                                                                                                                                                                                                                      |
|---------------|------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| 12:00         | Call to Order, Roll Call, and Welcome                                                                                                                                                                                                                                                                                                                            |
| 12:00 – 12:05 | <b>Consent Agenda:</b> <ul style="list-style-type: none"> <li>Minutes of the July 19, 2016 Board Meeting</li> <li>Rare Disease Advisory Panel (RDAP) Charter Revisions</li> </ul>                                                                                                                                                                                |
| 12:05 – 12:20 | <b>Consider for Approval:</b> New Advisory Panelists and Chairs                                                                                                                                                                                                                                                                                                  |
| 12:20 – 12:30 | <b>Consider for Approval:</b> PCORnet PPRN Demonstration Project                                                                                                                                                                                                                                                                                                 |
| 12:30 – 1:30  | <b>Report from the Science Oversight Committee:</b><br>Enhanced Funding Opportunities<br><b>Consider for Approval:</b> <ul style="list-style-type: none"> <li>Re-opening of selected Targeted PFAs</li> <li>Special emphasis topics for PCS PFA</li> </ul> <b>For Discussion:</b><br>Process for Reopening Targeted PFAs and Special Emphasis Topics in PCS PFAs |
| 1:30          | Wrap up and Adjournment                                                                                                                                                                                                                                                                                                                                          |



# Consent Agenda Items

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**Grayson Norquist, MD, MSPH**

Chairperson, Board of Governors

**Joe Selby, MD, MPH**

Executive Director



# Motion for Consent Agenda Items

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**That the Board approve:**

- Minutes from the July 19, 2016 Board meeting
- Proposed amended Charter for the Rare Disease Advisory Panel (RDAP)



# Board Vote

## Call for a Motion to:

- **Approve** each of the Motions on the Consent Agenda

## Call for the Motion to Be Seconded:

- **Second** the Motion
  - If further discussion, may propose an **Amendment** to the Motion or an **Alternative Motion**

## Voice Vote:

- Vote to **Approve** the **Final** Motion
  - Ask for votes in favor, opposed, and abstentions



# Approving New and Reappointed Advisory Panelists

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**Evelyn P. Whitlock, MD, MPH**

Chief Science Officer



# Topics and Objectives

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## **Advisory Panels Application and Selection Process Overview**

- Key information regarding the openings and applications received in this cycle of selection

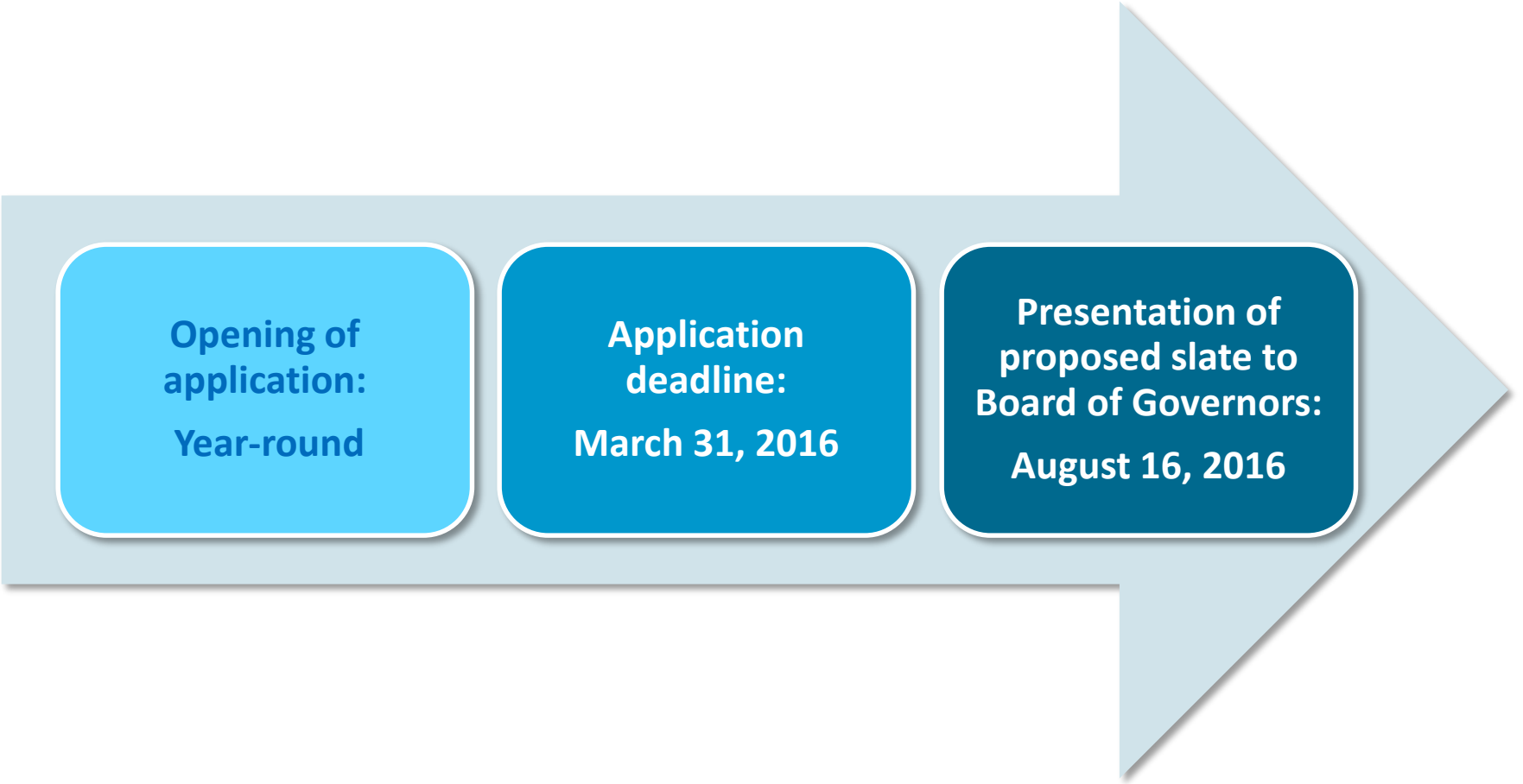
## **Proposed Advisory Panel Members**

- Review and approve proposed Advisory Panel members



# Timeline

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**Opening of  
application:  
Year-round**

**Application  
deadline:  
March 31, 2016**

**Presentation of  
proposed slate to  
Board of Governors:  
August 16, 2016**



# Openings and Applications Received

| Advisory Panel                                              | Open Positions | Applications Received |
|-------------------------------------------------------------|----------------|-----------------------|
| Assessment of Prevention, Diagnosis, and, Treatment Options | 7              | 99                    |
| Improving Healthcare Systems                                | 8              | 145                   |
| Addressing Disparities                                      | 7              | 81                    |
| Patient Engagement                                          | 8              | 140                   |
| Clinical Trials                                             | 10             | 89                    |
| Rare Disease                                                | 4              | 31                    |
| Communication and Dissemination Research                    | 1              | 49                    |
| <b>TOTAL</b>                                                | <b>45</b>      | <b>405*</b>           |

\*Applicants were able to apply to a maximum of two Advisory Panels, which is why this number does not equal the sum of the applications received



# 3<sup>rd</sup> Party Nominations Received From:

- Academy of Managed Care Pharmacy
- Academy of Radiology Research
- American Academy of Family Physicians
- American Academy of Hospice and Palliative Medicine
- American Academy of Nursing
- American Academy of Nursing and American Association of Colleges of Nursing
- American Academy of Orthopaedic Surgeons
- American Academy of Pediatrics
- American Academy of Physical Medicine and Rehabilitation
- American Association of Colleges of Nursing
- American Association of Neuroscience Nurses
- American Association of Nurse Anesthetists
- American Chiropractic Association
- American College of Physicians
- American College of Radiology
- American Epilepsy Society
- American Medical Society for Sports Medicine
- American Optometric Association
- American Osteopathic Association
- American Physical Therapy Association
- American Psychological Association
- American Society for Radiation Oncology
- American Society of Health-System Pharmacists
- American Society of Nephrology
- American Urological Association
- Asian & Pacific Islander American Health Forum
- Biotechnology Innovation Organization
- Celiac Disease Foundation
- Council of Academic Family Medicine
- Emergency Nurses Association
- Healthwise
- Medical Imaging & Technology Alliance
- Michigan Breast Cancer Coalition
- National Association of Pediatric Nurse Practitioners
- National Health Council
- National Kidney Foundation
- National Multiple Sclerosis Society
- North American Spine Society
- Oncology Nursing Society
- Society of Interventional Radiology
- Society of Thoracic Surgeons



# Advisory Panel on Addressing Disparities: Proposed Panelists and Alternates

| Name             | Stakeholder Group | Primary Affiliation                                        | Term (in Years) |
|------------------|-------------------|------------------------------------------------------------|-----------------|
| Terrie Black     | Clinician         | The University of Massachusetts & Joint Commission         | 3               |
| Deidra Crews*    | Clinician         | Johns Hopkins University School of Medicine                | 3               |
| Christine Joseph | Researcher        | Henry Ford Health System                                   | 3               |
| Donald Klepser*  | Researcher        | University of Nebraska Medical Center                      | 3               |
| Ana Maria Lopez* | Clinician         | University of Utah Health Sciences                         | 3               |
| Umbereen Nehal   | Payer             | University of Massachusetts/MassHealth (Medicaid)          | 3               |
| Tung Nguyen      | Researcher        | University of California, San Francisco School of Medicine | 3               |

\*Proposed panelists that were nominated by a third party organization have been marked with an asterisk



# Advisory Panel on Addressing Disparities: Proposed Panelists and Alternates

| Name                       | Stakeholder Group          | Primary Affiliation                | Term (in Years) |
|----------------------------|----------------------------|------------------------------------|-----------------|
| Alternates                 |                            |                                    |                 |
| Tamarah Deuperval-Brownlee | Hospital/<br>Health System | Ascension Health                   | 3               |
| New Chair and Co-Chair     |                            |                                    |                 |
| Cheryl Pegus               | Clinician                  | NYU Langone Medical<br>Center      | 2               |
| Elizabeth A. Jacobs        | Researcher                 | University of<br>Wisconsin-Madison | 1               |



# Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options: Proposed Panelists and Alternates

| Name                       | Stakeholder Group                      | Primary Affiliation                                             | Term (in Years) |
|----------------------------|----------------------------------------|-----------------------------------------------------------------|-----------------|
| Zeeshan Butt*              | Researcher                             | Northwestern University                                         | 3               |
| Rafael Alfonso-Cristancho* | Industry                               | GlaxoSmithKline                                                 | 3               |
| Felix Fernandez*           | Clinician                              | Atlanta VA Medical Center & Emory University School of Medicine | 3               |
| Jeff Hersh*                | Industry                               | GE Health Care                                                  | 3               |
| Susan Lin                  | Patient/Caregiver/<br>Patient Advocate | American Occupational Therapy Association                       | 3               |
| Michael Schneider*         | Researcher                             | University of Pittsburgh                                        | 3               |
| Nancy White*               | Clinician                              | Arlington Free Clinic                                           | 3               |

\*Proposed panelists that were nominated by a third party organization have been marked with an asterisk



# Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options: Proposed Panelists and Alternates

| Name                        | Stakeholder Group    | Primary Affiliation                                             | Term (in Years) |
|-----------------------------|----------------------|-----------------------------------------------------------------|-----------------|
| <b>Alternate Candidates</b> |                      |                                                                 |                 |
| Cheryl Holly                | Training Institution | Rutgers University School of Nursing                            | 3               |
| Elaine Larson*              | Researcher           | Columbia University School of Nursing & School of Public Health | 3               |
| Jeff Quinlan*               | Clinician            | Uniformed Services University of the Health Sciences            | 3               |
| <b>New Chair</b>            |                      |                                                                 |                 |
| Margaret Clayton            | Researcher           | University of Utah                                              | 1               |

\*Proposed panelists that were nominated by a third party organization have been marked with an asterisk



# Advisory Panel on Clinical Trials: Proposed Panelists and Alternates

| Name               | Stakeholder Group                      | Primary Affiliation                            | Term (in Years) |
|--------------------|----------------------------------------|------------------------------------------------|-----------------|
| Kaleab Abebe*      | Researcher                             | University of Pittsburgh<br>School of Medicine | 3               |
| Patrick Archdeacon | Researcher                             | US Food and Drug<br>Administration             | 3               |
| Seth Morgan*       | Patient/Caregiver/<br>Patient Advocate | N/A                                            | 3               |
| Carole Treston     | Researcher                             | Association of Nurses in<br>AIDS Care          | 3               |

\*Proposed panelists that were nominated by a third party organization have been marked with an asterisk



# Advisory Panel on Clinical Trials: Proposed Panelists and Alternates

| Name                        | Stakeholder Group                      | Primary Affiliation                            | Term (in Years) |
|-----------------------------|----------------------------------------|------------------------------------------------|-----------------|
| <b>Alternate Candidates</b> |                                        |                                                |                 |
| Mei Lu                      | Researcher                             | Henry Ford Health System                       | 3               |
| Karen Jackson*              | Patient/Caregiver/<br>Patient Advocate | Retired                                        | 3               |
| Jeffery Jarvik*             | Clinician                              | University of Washington School of<br>Medicine | 3               |
| John Laschinger*            | Researcher                             | US Food and Drug Administration                | 3               |

\*Proposed panelists that were nominated by a third party organization have been marked with an asterisk



# Communication and Dissemination Research: Proposed Panelists and Alternates

| Name                        | Stakeholder Group                  | Primary Affiliation                     | Term (in Years) |
|-----------------------------|------------------------------------|-----------------------------------------|-----------------|
| Emilie Johnson*             | Clinician                          | Lurie Children's Hospital of Chicago    | 3               |
| <b>Alternate Candidates</b> |                                    |                                         |                 |
| Marta Brooks                | Clinician                          | Regis University                        | 3               |
| Elizabeth Krause*           | Patient/Caregiver/Patient Advocate | Connecticut Health Foundation           | 3               |
| <b>Reappointed Members</b>  |                                    |                                         |                 |
| Ashish Atreja               | Researcher                         | Icahn School of Medicine at Mount Sinai | 3               |
| Danny van Leeuwen           | Patient/Caregiver/Patient Advocate | Advocates, Inc.                         | 3               |
| Nancy Perrin                | Researcher                         | Kaiser Permanente Northwest             | 3               |
| Janice Radak                | Patient/Caregiver/Patient Advocate | American Heart Association              | 3               |
| Frank Rider                 | Patient/Caregiver/Patient Advocate | American Institutes for Research        | 3               |
| Kristin Voorhees            | Patient/Caregiver/Patient Advocate | Beyond Celiac                           | 3               |

\*Proposed panelists that were nominated by a third party organization have been marked with an asterisk



# Advisory Panel on Improving Healthcare Systems: Proposed Panelists and Alternates

| Name              | Stakeholder Group                      | Primary Affiliation                                    | Term (in Years) |
|-------------------|----------------------------------------|--------------------------------------------------------|-----------------|
| Rebecca Aslakson* | Researcher                             | Johns Hopkins School of Medicine                       | 3               |
| Leah Backhus*     | Clinician                              | Veterans Affairs and Stanford University               | 3               |
| Ignatius Bau*     | Patient/Caregiver/<br>Patient Advocate | N/A                                                    | 3               |
| James Perrin*     | Researchers                            | Massachusetts General Hospital Physician Organization  | 3               |
| Alexis Snyder*    | Patient/Caregiver/<br>Patient Advocate | N/A                                                    | 3               |
| Craig Umscheid*   | Hospital/Health System                 | University of Pennsylvania Perelman School of Medicine | 3               |
| Mitzi Wasik*      | Payer                                  | Aetna                                                  | 3               |
| Nancy Yedlin      | Researcher                             | Donaghue Foundation                                    | 3               |

\*Proposed panelists that were nominated by a third party organization have been marked with an asterisk



# Advisory Panel on Improving Healthcare Systems: Proposed Panelists and Alternates

| Name                        | Stakeholder Group                    | Primary Affiliation                                            | Term (in Years) |
|-----------------------------|--------------------------------------|----------------------------------------------------------------|-----------------|
| <b>Alternate Candidates</b> |                                      |                                                                |                 |
| Marian Grant                | Clinician                            | Coalition to Transform Advanced Care                           | 3               |
| Amy Houtrow*                | Researcher                           | University of Pittsburgh School of Medicine                    | 3               |
| Jacqueline Merrill*         | Clinician                            | Columbia University School of Nursing                          | 3               |
| Elizabeth Rubinstein        | Patient/ Caregiver/ Patient Advocate | N/A                                                            | 3               |
| Kimberly Saverno*           | Researcher                           | Humana                                                         | 3               |
| <b>New Co-Chair</b>         |                                      |                                                                |                 |
| Timothy Daaleman            | Researcher                           | University of North Carolina at Chapel Hill School of Medicine | 1               |

\*Proposed panelists that were nominated by a third party organization have been marked with an asterisk



# Advisory Panel on Patient Engagement: Proposed Panelists and Alternates

| Name                        | Stakeholder Group                      | Primary Affiliation                        | Term (in Years) |
|-----------------------------|----------------------------------------|--------------------------------------------|-----------------|
| John Chernesky              | Patient/Caregiver/<br>Patient Advocate | Rick Hansen Institute                      | 2               |
| Emily Creek                 | Patient/Caregiver/<br>Patient Advocate | Arthritis Foundation                       | 2               |
| Libby Hoy                   | Patient/Caregiver/<br>Patient Advocate | Patient & Family Centered<br>Care Partners | 2               |
| Megan Lewis                 | Researchers                            | RTI International                          | 2               |
| Suzanne Madison             | Patient/Caregiver/<br>Patient Advocate | N/A                                        | 2               |
| Ting Pun                    | Patient/Caregiver/<br>Patient Advocate | N/A                                        | 2               |
| John Westfall*              | Clinician                              | Colorado HealthOP                          | 2               |
| David White                 | Patient/Caregiver/<br>Patient Advocate | N/A                                        | 2               |
| <b>Alternate Candidates</b> |                                        |                                            |                 |
| Michael Siegel              | Patient/Caregiver/<br>Patient Advocate | National Psoriasis<br>Foundation           | 2               |

\*Proposed panelists that were nominated by a third party organization have been marked with an asterisk



# Advisory Panel on Rare Disease: Proposed Panelists and Alternates

| Name              | Stakeholder Group                      | Primary Affiliation                                     | Term (in Years) |
|-------------------|----------------------------------------|---------------------------------------------------------|-----------------|
| Matt Cheung       | Industry                               | University of the Pacific                               | 3               |
| Kathleen Gondek   | Industry                               | Shire PLC                                               | 3               |
| Michael Kruer*    | Researcher                             | Phoenix Children's Hospital                             | 2               |
| John Mulvihill    | Clinician                              | University of Oklahoma<br>College of Medicine           | 2               |
| Maureen Smith     | Patient/Caregiver/<br>Patient Advocate | N/A                                                     | 3               |
| Penelope Strauss* | Training Institution                   | University of Texas Health<br>Science Center at Houston | 1               |
| <b>Alternate</b>  |                                        |                                                         |                 |
| John Gearhart*    | Clinician                              | Johns Hopkins University<br>School of Medicine          | 3               |

\*Proposed panelists that were nominated by a third party organization have been marked with an asterisk



# Board Vote

## Call for a Motion to:

- **Approve** the proposed slates of new and reappointed members and terms for the following Advisory Panels: APDTO, IHS, AD, PEAP, CTAP, RDAP, CDR

## Call for the Motion to Be Seconded:

- **Second** the Motion
  - If further discussion, may propose an **Amendment** to the Motion or an **Alternative Motion**

## Voice Vote:

- Vote to **Approve** the **Final** Motion
  - Ask for votes in favor, opposed, and abstentions



# Consider for Approval: PCORnet Patient-Powered Research Networks (PPRN) Research Demonstration Project

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**Rachael Fleurence, PhD**

Program Director, Research Infrastructure, PCORI



# Project Background

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- PCORI sought to fund PPRN-initiated research based on questions that have been generated and prioritized by participants within the PPRN community.
- **Project Title:** Using Single Subject (N-of-1) Designs to Answer Patient-Identified Research Questions
- **Summary of Research Project:** tech-enabled N-of-1 trials will be used to help answer patient-generated research questions among children 0-18 with Inflammatory Bowel Disease (IBD) and adults (18+) with Paroxysmal Atrial Fibrillation (AF)
- **Collaborating** with 1 other PPRN
- **Outcomes** will include:
  - IBD: stool and pain; patient reported outcomes; intestinal inflammation
  - AF: change in AF severity; AF frequency
- **Specific Aims:**
  - Compare the effectiveness of the Specific Carbohydrate Diet (SCD) in patients with IBD versus a liberalized SCD or an unrestricted diet
  - Compare N-of-1 trials versus data tracking to identify and eliminate individual-level triggers and reduce AF frequency and severity
- This project was approved by the Selection Committee on August 9, 2016



# Slate Summary

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| Total Budget | Project Focus                                                                         |
|--------------|---------------------------------------------------------------------------------------|
| \$2.5M       | Using Single Subject (N-of-1) Designs to Answer Patient-Identified Research Questions |

*\*All proposed projects, including requested budgets and project periods, are approved subject to a programmatic and budget review by PCORI staff and the negotiation of a formal award contract.*



# Contributions to Infrastructure Development

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| Project                                     | Contributions to the PCORnet Commons                                                                                                                                                                        |
|---------------------------------------------|-------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| <b>N-of-1 Trials in IBD and AF Patients</b> | <ul style="list-style-type: none"><li>• N-of-1 Trials methodology to provide personalized information for care choices</li><li>• Robust procedures and processes to enable N-of-1 experimentation</li></ul> |



# Slate Overview:

*Patient-Powered Research Networks  
Research Demonstration Projects*

Approved  
5  
Projects

Proposed  
5 + 1 = 6  
Projects

| PFA                                                                                  | Allotted | Previously<br>Awarded<br>Budget | Proposed<br>Total Budget* | Average<br>Project<br>Budget* |
|--------------------------------------------------------------------------------------|----------|---------------------------------|---------------------------|-------------------------------|
| Patient-Powered<br>Research Networks<br>(PPRN) Research<br>Demonstration<br>Projects | \$18M    | \$12.5M                         | \$15M                     | \$2.5M                        |

\*Total budget = direct + indirect costs

*\*All proposed projects, including requested budgets and project periods, are approved subject to a programmatic and budget review by PCORI staff and the negotiation of a formal award contract.*



# Board Vote

## Call for a Motion to:

- **Approve** funding for the recommended additional Patient-Powered Research Networks (PPRN) Demonstration Project Award

## Call for the Motion to Be Seconded:

- **Second** the Motion
  - If further discussion, may propose an **Amendment** to the Motion or an **Alternative Motion**

## Roll Call Vote:

- Vote to **Approve** the **Final** Motion
  - Ask for votes in favor, opposed, and abstentions



# Enhanced Funding Opportunities: Report from the Science Oversight Committee (SOC)

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**Robert Zwolak, MD, PhD**

Science Oversight Committee Chair

**Evelyn P. Whitlock, MD, MPH**

Chief Science Officer



# SOC Developing Suite of Strategic Funding Opportunities

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- A shortage of meritorious applications left un-awarded funds during recent cycles
- Expanded strategic funding opportunities will benefit PCORI and researcher applicants
- We are presenting two SOC-approved opportunities today for discussion and approval
- We will outline for the Board additional SOC-developed strategic funding initiatives in the near-term



# New SOC-developed Strategic Funding Initiatives Build Upon Established Board Processes

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- Responding to Board suggestions and internal SOC work, we have developed two new strategies that Evelyn will discuss:
  - Re-opening of selected targeted PFAs
  - Calling out Special Emphasis topics within PCS PFAs
- These SOC-developed strategies build upon established Board processes
- We are bringing SOC-approved PFAs representing both new initiatives for a vote today
- Once the Board votes, we will discuss the process by which SOC and staff will handle these initiatives going forward



# Enhanced Funding Opportunities: Presentation for Approval

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**Evelyn P. Whitlock, MD, MPH**  
Chief Science Officer

**Robert Zwolak, MD, PhD**  
Science Oversight Committee Chair



# Pathway to a Funding Announcement

Staff use Tier 1 and Tier 2 review criteria to determine topic eligibility, **producing List 1**

Science Oversight Committee (SOC) reviews and endorses topics for topic briefs, **producing List 2**

SOC reviews topic briefs and approves them for Advisory Panel review, **producing List 3**

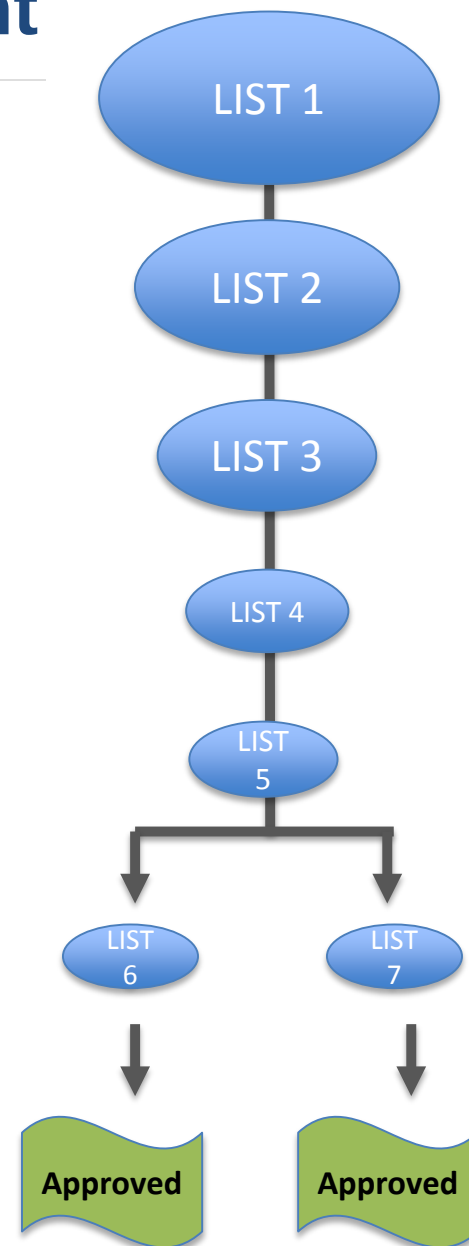
Advisory Panel (AP) reviews topic briefs using Tier 3 review criteria, **producing List 4**

SOC reviews AP results and staff recommendations; endorses topics for further refinement, **producing List 5**

Staff and SOC use Tier 4 review criteria to assess questions; SOC assigns questions to targeted or Pragmatic Clinical Studies PFA, **producing Lists 6 and 7**

Board reviews and approves questions for targeted PFA

SOC reviews and approves questions for Pragmatic Clinical Studies PFA



# New Strategic Funding Initiatives Complement and Extend Current Funding Opportunities

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- We have an established Pathway for Funding Announcements (in addition to Broad program)
- These result in targeted PFAs and PCS announcements at regular funding cycles
- The SOC-recommended two strategic initiatives build on this process to augment PCORI's awarding of meritorious priority topics
  - Re-opening of targeted PFAs where appropriate
  - Calling out PCS Special Emphasis topics



# Enhanced Funding Opportunities: Re-opening Recent Targeted PFAs Cycle 3, 2016

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**Evelyn P. Whitlock, MD, MPH**  
Chief Science Officer

**Robert Zwolak, MD, PhD**  
Science Oversight Committee Chair



# How Re-opening Targeted PFAs Builds on Existing Processes

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- The targeted PFA process (List 6) has resulted in 12 targeted PFAs with 3 more planned for August 2016
- For the 4 targeted PFAs posted in October 2015 (awarded in July 2016), we did not allocate all of the approved funds
- The SOC has approved re-opening up to 3 targeted PFAs from the October 2015 cycle for which there are remaining studies to be conducted and approved dollars to be awarded



# The Board asked SOC to Assess Re-opening Targeted PFAs

| 2015 Cycle 3 Targeted PFAs                  | # of Research Questions | # of Projects Awarded/ Estimated | Budget Awarded/ Posted | Re-opening Opportunities                  |
|---------------------------------------------|-------------------------|----------------------------------|------------------------|-------------------------------------------|
| New Oral Anticoagulants                     | 1                       | 2/3                              | \$6.5/\$30M            | Add RCT study design                      |
| Long-Term Opioid Treatment for Chronic Pain | 2                       | 2/2                              | \$21/\$40M             | More studies needed in high-priority area |
| Treatment of Multiple Sclerosis             | 3                       | 4/8                              | \$19.6/\$50M           | More studies needed in high-priority area |

**RCT: Randomized Controlled Trial**

- Science Oversight Committee recommends re-opening up to 3 targeted PFAs from the October 2015 cycle, based on achieving additional important research goals and available approved funds
- Timing should allow additional awards within FY2017



# Board Vote

Call for a Motion to:

- **Approve** re-opening of one or more of the Cycle 3 2015 targeted PFAs (NOACs in the Extended Treatment of Venous Thromboembolic Disease, Long-Term Opioid Treatment for Chronic Pain, Treatment of Multiple Sclerosis) for up to \$73M remaining from the previously approved budgets

Call for the Motion to Be Seconded:

- **Second** the Motion
  - If further discussion, may propose an **Amendment** to the Motion or an **Alternative Motion**

Roll Call Vote:

- Vote to **Approve** the **Final** Motion
  - Ask for votes in favor, opposed, and abstentions



# Enhanced Funding Opportunities: Special Emphasis Pragmatic Clinical Studies Topics Cycle 3 2016

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**Evelyn P. Whitlock, MD, MPH**  
Chief Science Officer

**Robert Zwolak, MD, PhD**  
Science Oversight Committee Chair



# How PCS Special Emphasis Strategy Builds on Existing Processes

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- The priority topics pathway for Pragmatic Clinical Studies (PCS) has resulted in 20 currently active topics (List 7); these were recently re-ratified by the SOC for the Cycle 3 2016 PFA releases
- In April 2016, the SOC approved an additional strategy for PCS PFA called **Special Emphasis**
  - Selected topics are listed with set-aside funds, using monies already approved for PCS but not awarded
  - These do not detract from existing PCS PFA opportunities nor require additional funding



# SOC-approved Special Emphasis topics

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- The SOC undertook an explicit prioritization and evaluation process for considering PCS topics for Special Emphasis
  - Topic genesis and history was reviewed, including stakeholder priorities
  - Previous proposed research (LOIs and proposals)
  - Representation of topic in PCORI funded portfolio
  - Current evidence gaps and research needs updated
- After SOC evaluation, two Special Emphasis topics were selected:
  - Community-acquired Pneumonia
  - Pelvic Organ Prolapse
- Science Oversight Committee approved including these as Special Emphasis topics for Cycle 3 2016 PCS PFA



# Community Acquired Pneumonia – Topic History

**Topic Origin:** American College of Physicians – Clinical Guideline Committee (2014)

**Advisory Panel Review:** APDTO; October 9, 2015

**PCS Priority Topic History:** Approved as a PCS priority topic on March 15, 2016

- Listed as a PCS priority topic in 1 cycle

## **Current Priority Topic:**

- What is the comparative effectiveness and safety of alternative antibiotic regimens in the empiric outpatient treatment of adults with community-acquired pneumonia?
  - Varying durations of treatment, such as 3 or 5 days, or discontinuation at resolution of symptoms of infection
  - Across-class comparisons of FDA-approved antibiotic regimens that are recommended for use or are commonly used in community-acquired pneumonia
- Studies should address effectiveness in distinct subpopulations (e.g., patients with chronic conditions, immunosuppression, and the elderly)

## **Additional Interested Stakeholders:**

- Infectious Disease Society of America (antimicrobial stewardship, 2016)
- Premier Inc. (antimicrobial stewardship, 2014)



# Community Acquired Pneumonia—Background

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- Antibiotics are standard treatment for CAP; however, they are associated with the development of bacterial resistance and result in patient side effects
- **IDSA/ATS 2007** guidelines most commonly used (2017 summer update planned):
  - **Drug type:**
    - Previously healthy: Use a macrolide or doxycycline
    - Comorbidities: Use a fluoroquinolone or a  $\beta$ -lactam + macrolide
  - **Duration:**  $\geq 5$  days, if afebrile for 48-72 hours, and no more than 1 CAP-associated sign of clinical instability
- Existing evidence:
  - **Drug type:** 2014 Cochrane review concludes evidence too limited to recommend a single best class; notes important differences in adverse effects between classes (e.g., GI, nervous system)
  - **Duration:** 2008 Cochrane review concludes long courses of antibiotics (14 days) no more effective than shorter (7-10 day); no evidence on more abbreviated courses



# Community Acquired Pneumonia – New Research

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- Limited new research since the 2015 topic brief:
  - **Drug type:**
    - Claims-based retrospective cohort study in Taiwan comparing penicillin v. fluoroquinolones in adult outpatient clinics:
      - Marginally lower likelihood of treatment failure with fluoroquinolones; no differences in hospitalizations, ER visits, or 30-day mortality
    - 2015 *NEJM* inpatient RCT in adults with suspected CAP (CAP-START):
      - Beta-lactam monotherapy noninferior to either fluoroquinolone monotherapy or beta-lactam-macrolide combination therapy for 90-day mortality
  - **Duration:**
    - July 2016 *JAMA Internal Medicine* inpatient RCT in adults with CAP:
      - Adults meeting IDSA/ATS clinical stability criteria with antibiotic treatment discontinued after 5 days had similar rates of resolution of infection 30 days after infection compared with usual care
- Paucity of ongoing **head-to-head pharmaceutical** research and initial positive findings with regards to **shorter duration therapy** in different care settings suggest both are ripe areas for additional study at this time



# Pelvic Organ Prolapse – Topic History

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**Topic Origin:** Multiple topics submitted regarding the safety of transvaginal mesh (Web portal, 2014)

**Advisory Panel Review:** APDTO; January 14, 2014

**PCS Priority Topic History:** Approved as PCS priority topic on August 8, 2014

- Listed as PCS priority topic in 4 cycles

## **Current Priority Topic:**

- Compare the relative benefits and harms of different types of current FDA-approved transvaginal mesh (e.g., permanent vs. absorbable) or biological grafts in repairing pelvic organ prolapse in terms of infections, de novo urinary or fecal incontinence, bowel injury, pain, sexual function, quality of life, and other patient-centered outcomes
- Compare the relative benefits and harms of different surgical techniques in repairing pelvic organ prolapse by compartment (i.e., anterior, posterior, multi-compartment) using currently FDA-approved transvaginal mesh in terms of the outcomes mentioned above



# Pelvic Organ Prolapse – Current State of Evidence

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- **FDA regulations:**
  - In January 2016, pelvic mesh for pelvic organ prolapse (POP) reclassified as a class III high-risk device; increased reports of adverse events associated with transvaginal repair using surgical mesh
- Potential **benefits** of transvaginal mesh repair of POP:
  - Lower rates of awareness of prolapse and repeat surgery compared to native repair
  - Reduced risk of recurrent prolapse
  - May be limited to the anterior compartment (versus apical or posterior)
- Potential **harms** of transvaginal mesh repair of POP:
  - Higher surgical complication rates: mesh exposure, de novo stress urinary incontinence, bladder injury, pelvic pain, voiding dysfunction, dyspareunia
- Although alternative methods such as **absorbable mesh or biological grafts** may have the potential to improve the risk-benefit profile of POP repair, available evidence is **too limited** to draw any conclusions



# Approach to PCS Special Emphasis Topics

- Posted within usual PCS PCORI Funding Announcement (one-time)
- Areas of Special Emphasis indicated in written text of PFA as well as supportive text boxes
- Outreach planned
- Set-aside funding taken from uncommitted PCS funds from FY2016 funding announcements

|                       | PCS Special Area of Emphasis |                       |
|-----------------------|------------------------------|-----------------------|
|                       | Community-acquired Pneumonia | Pelvic Organ Prolapse |
| Total Funds Available | Up to \$40M                  | Up to \$40M           |



# Board Vote

## Call for a Motion to:

- **Approve** designating two special emphasis topics (Community-acquired Pneumonia and Pelvic Organ Prolapse) in a future PCS PFA with set aside funding of up to \$80M remaining from previously approved PCS budgets

## Call for the Motion to Be Seconded:

- **Second** the Motion
  - If further discussion, may propose an **Amendment** to the Motion or an **Alternative Motion**

## Roll Call Vote:

- Vote to **Approve** the **Final** Motion
- Ask for votes in favor, opposed, and abstentions



# Board Processes for Re-opening Targeted PFAs and PCS Special Emphasis Topics

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**Robert Zwolak, MD, PhD**

Science Oversight Committee Chair



# How Re-opening Targeted PFAs Builds on Existing Processes

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- The targeted PFA process is valuable but resource intensive, and has resulted in 12 targeted PFAs with 3 more planned for August 2016
- For recent targeted PFAs, we did not allocate all of the approved funds
- Going forward, the SOC proposes having the option to reopen some or all of the questions/targeted PFAs for which there are remaining studies to be conducted and approved dollars to be awarded



# How PCS Special Emphasis Strategy Builds on Existing Processes

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- Priority topics for Pragmatic Clinical Studies is an important research funding strategy the Board has developed
- About 50% of funded PCS studies represent our PCS topics
- Recent PCS PFAs did not result in a sufficient volume or range of meritorious proposals
- The SOC has approved an additional strategy for PCS PFA called **Special Emphasis** to enhance the current PCS process
  - Selected topics are listed with set-aside funds, using moneys already approved for PCS but not awarded
  - These do not detract from existing PCS PFA opportunities nor require additional funding



## Discussion of Future Processes for Re-opening Targeted PFAs and PCS Special Emphasis Topics

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***Discuss and provide direction for drafting policy related to whether and under what circumstances decisions about the following matters should be delegated to the Science Oversight Committee (SOC):***

- Re-opening targeted PFAs when Board-approved funds remain after the initial cycle of funding
- Designating Board-approved funds that remain from previous cycles of PCS PFAs for Special Emphasis Topics in subsequent PCS cycles



# Wrap Up and Adjournment

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**Grayson Norquist, MD, MSPH**

Chairperson, Board of Directors

