

# Research Prioritization Breakout

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Advisory Panel on Rare Disease Winter 2016 Breakout Webinars  
January 27, 2016



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# Welcome and Agenda

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**Marilyn Bull, MD, FAAP**

Member, Advisory Panel on Rare Disease, PCORI

**Parag Aggarwal, PhD**

Senior Program Officer, Addressing Disparities, PCORI



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# Housekeeping

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- 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.
- Anyone may submit a comment through the webinar chat function or by emailing [advisorypanels@pcori.org](mailto:advisorypanels@pcori.org).
- Visit [www.pcori.org/events](http://www.pcori.org/events) for more information.
- Chair Statement on COI and Confidentiality

# Today's Agenda

Start Time	Item	Speaker
12:30 p.m.	Welcome and Agenda	M. Bull P. Aggarwal
12:45 p.m.	Literature Review Summary and Discussion	P. Aggarwal
1:15 p.m.	Outline Review	
2:15 p.m.	Recap and Next Steps	M. Bull P. Aggarwal
2:30 p.m.	Break	

# Background

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- During RDAP Spring meeting topics missing in the landscape review were identified to be addressed in a follow up document
- PCORI staff called for volunteers for each topic; 4 topics were covered by volunteers:
  - Human Subjects
  - Incorporating PROs into Registries
  - Registry Purposes
  - Evidence Grading
- PCORI staff/RDAP leadership proposed a reframing of the priority topics

# Proposed Reframing of Priority Topics for Further Guidance

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- Human subject issues specific to rare diseases
- The importance of and best practices for research prioritization
- Considerations related to the challenges with producing reliable evidence for rare diseases

# Breakouts and Participants

- **Human Subjects**

- Patricia Furlong (chair)
- Kate Lorig
- Sindy Escobar-Alvarez
- Philip Ruff

- **Research Prioritization**

- Marilyn Bull (chair)
- Jacqueline Alikhaani
- Vincent Del Gaizo
- Mardi Gomberg-Maitland
- Lisa Heral
- William Whitehead

- **Challenges with Producing Reliable Evidence for Rare Diseases**

- Naomi Aronson (chair)
- Yaffa Rubinstein
- Marshall Summar
- Mark Skinner
- James Wu

# Breakout 2: Research Prioritization – Key Questions

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- What are some good examples of a cohesive rare disease research community that was able to come to consensus regarding the research priorities for a given rare condition?
- What are some best practices to engage the patient and stakeholder community in setting a research agenda?
- What are some best practices to form strong partnerships between the rare disease patient/caregiver communities and the research communities, to ensure that the priorities established are implemented?



# Project Timeline

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- **November 2015 – January 2016:** Refine the workgroup objectives and deliverables and develop an outline for the workgroup document. At the January 2016 RDAP meeting, time will be reserved for workgroups to meet and review their document outlines.
- **January 2016 – April 2016:** Draft a document directed at the rare disease community based on the outline discussed at the January 2016 RDAP meeting. At the April 2016 RDAP meeting, time will be reserved for the workgroups to discuss the complete draft documents.
- **April 2016 – July 2016:** Revise and finalize the draft document. Time will be reserved at the July 2016 RDAP meeting for presentations of the final documents. The goal is to publish the documents produced by each group on the PCORI website and in a special issue of a peer-reviewed medical journal.



# Literature Review Summary and Discussion

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Parag Aggarwal, PhD

Senior Program Officer, Addressing Disparities, PCORI



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# Final Search Strategy

- ("rare diseases"[tw] OR "rare disease"[tw]) AND ("topic prioritization"[tw] OR "topic development" [tw] OR "topic identification" [tw] OR "topic refinement" [tw] OR "priority topics" [tw] OR "topic nomination"[tw] OR "setting priorities" [tw] OR "priority setting" [tw] OR "developing priorities" [tw] OR "priority areas" [tw] OR "research priorities"[tw] OR "research priorities" [tw] OR "research prioritization" [tw] OR "prioritization"[tw] OR "research agenda" [tw] OR "agenda setting" [tw] OR "question prioritization" [tw] OR "question development" [tw] OR "question identification" [tw] OR "question refinement" [tw] OR "priority questions" [tw] OR "prioritizing research"[tw] OR "prioritizing questions"[tw] OR "research needs" [tw] OR "research planning" [tw] OR "research collaboration"[tw] OR "collaborative research"[tw] OR "funding research"[tw]) AND (("2003/01/01"[PDAT] : "3000/12/31"[PDAT]) AND English[lang])
- o Articles Returned: 47

# Discussion Questions

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- Is there anything missing from our search strategy?
- Are there any important articles missing?
- What are your overall thoughts on the literature search and the results?

# Outline Review

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# Discussion Questions

- Should we structure the outline to follow the best practices throughout different stages of the research process (delineated in the objectives section of the breakout summary)? (e.g., creating a “roadmap” as discussed in the breakout session)
  - E.g., infancy to intermediate to well-defined
- Are the experts identified sufficient or do we need others? (next slide)
- Do we want to include data/information that is not specifically rare disease, but could help inform best practices?
- What do you envision the role of the expert writer to be?
  - Formatting and consolidating sections drafted by this group?
  - Drafting the whole document (need for specific expertise)?
  - Do you know potential candidates? PhD students?

# Experts to Engage

- Contacts at NORD
- Coordinating Center at George Washington University
- Paul Gross, founding partner of the Hydrocephalus Clinical Research Network (HCRN)
- Dr. Melissa Parisi
  - Eunice Kennedy Shriver National Institute of Child Health and Human Development, funded and developed the Down Syndrome registry.
  - NIH launches tool to advance Down syndrome research
- Contacts at Cornelia De Lange Syndrome Foundation
- Fanconi Anemia patient/advocacy group
  - American Society of Hematology
- Contacts at Adult Congenital Heart Association
- Muscle Atrophy patient/advocacy group
  - NORD Spinal Muscular Atrophy; Dr. Barry Russman
- PSCANNER in San Diego

# Recap and Next Steps

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# Next Steps

- Identification of any key articles to focus on (from the literature review or otherwise)
- Revision of outline based on today's discussion
- Identification and hiring of technical writer
- Meetings/interviews with experts
- Drafting of draft concept paper

# Thank You!



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