Human Subjects Breakout

Advisory Panel on Rare Disease Winter 2016 Breakout Webinars

January 27, 2016

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Welcome and Agenda

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Member, Advisory Panel on Rare Disease, PCORI

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Program Officer, Improving Healthcare Systems, PCORI

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

Today’s Agenda

Start Time	Item	Speaker
3:00 p.m.	Welcome and Agenda	P. Furlong G. Moscou-Jackson
3:15 p.m.	Literature Review Summary and Discussion	G. Moscou-Jackson
3:45 p.m.	Outline Review	
4:45 p.m.	Recap and Next Steps	P. Furlong G. Moscou-Jackson
5:00 p.m.	Break	

Background

- 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

- 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
  - James Wu
  - Marshall Summar
  - Mark Skinner

Breakout 1: Human Subjects – Key Questions

- What are the most important considerations when developing consent forms for registries enrolling adults with rare diseases?
- What are the most important considerations when developing consent forms for registries enrolling children with rare diseases?
- What measures can be implemented to protect the privacy of individuals who are enrolling in a rare disease registry?
- What are some best practices for developing consent forms and privacy protection measures for a rare disease registry?
- What are some best practices to engage patients, families, and/or caregivers in the development of consent forms and privacy protections measures for a rare disease registry?

Project Timeline

- **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

Gyasi Moscou-Jackson, PhD, MHS, RN

Program Officer, Improving Healthcare Systems, PCORI

Literature Review Search Strategy

- **Literature Review Question:**
  - What is the state of the science on informed consent in children and individuals with rare diseases (i.e., ethical concerns, best practices, and legal issues related to research participation and data sharing)?

MeSH Terms	Key Words
Informed Consent Informed Consent by Minors Parental consent/ethics Ethics, Research Biomedical Research/ethics Treatment Refusal/ethics Child Minors Cohort Studies	Consent, personal autonomy, privacy, confidentiality, child and adolescent rights Vulnerable Populations: rare disease, children, minors, young adults, competence/ incompetence Clinical trials, Cohort studies Ethics, Research Ethics Data sharing, data repository or repositories, data registries, biobanking

Literature Review Search Strategy

- **Search Limits:**
  - Publication year 2003 (‘Rare Diseases” Mesh term was introduced in 2003)
  - English language
- **Final Search Strategy**
  - ("Rare Diseases"[Mesh] OR "rare diseases"[tw] OR "rare disease"[tw] OR "rare disorders"[tw] OR "rare disorder"[tw] OR "rare conditions"[tw] OR "rare condition"[tw]) AND ("Informed Consent"[Mesh] OR "consent"[tw] OR "ethics"[tw] OR "ethical"[tw] OR "privacy"[tw] OR "personal autonomy"[tw] OR "confidentiality"[tw] OR "legal"[tw] OR "bioethical"[tw]) AND (("2003/01/01"[PDAT] : "3000/12/31"[PDAT]) AND English[lang])
    - Articles Returned: 260
    - Potentially Relevant Articles: 26 (identified by Gyasi via title and abstract review)

Discussion Questions

- What types of literature (e.g., commentary, case report, original research, etc.) should be included?
- Is the search strategy missing key words or MeSH terms?
- Were there any noteworthy articles that can be used to identify additional relevant articles and/or exemplify the review question?
- Are any important articles missing?
- What are your overall thoughts on the literature search and the results?

Outline Review

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Outline Review

- Context/Background
- Objective
- Data/Literature Sources
- Study Selection Criteria
- Data or Themes to be Extracted
- Results, Limitations, and Conclusions (discuss later)

Discussion Questions

- Are the experts identified sufficient or do we need others? (next slide)
- 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

- Donald Patrick, University of Washington, an expert in patient-reported outcomes who has a child with a rare disease
- Arthur Caplan, a bioethics expert at New York University
- An expert in government regulations pertaining to informed consent
- A parent of a child with a rare disease (such as Vincent Del Gaizo, the RDAP co-chair) who has discussed informed consent with that child
- An expert in informed consent (such as Dr. Yaffa Rubinstein, an RDAP member)
- Patients who have thought about informed consent for rare disease registries
- Society for Clinical Trials
- PCORI's Advisory Panel on Clinical Trials
- The Clinical Trials Transformation Initiative (CITTI), which has an informed consent project
- Pamela Tenaerts, Executive Director, is also on the Advisory Panel on Clinical Trials Expert Post-Award Subcommittee

Recap and Next Steps

Patricia Furlong

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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
- Potential journals
- Drafting of draft review paper

Thank You!

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