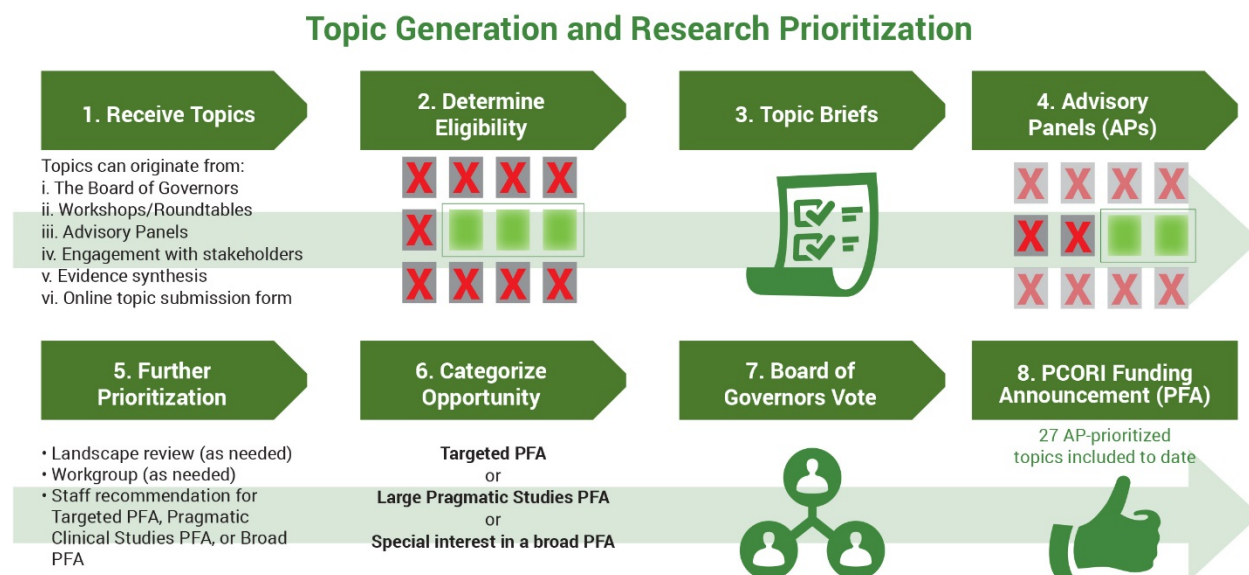# Advisory Panel on Rare Disease Fall 2015 Meeting: Research Prioritization Breakout

Background

The science of research prioritization has made important strides over the past two decades. From an approach largely driven by the research community, a number of research funding organizations such as AHRQ, PCORI, and the JLA have now established explicit processes that engage multi-stakeholder groups to conduct research topic prioritization.[1, 2] This trend mirrors a larger healthcare focus on patient and other stakeholder needs—which led to the creation of PCORI itself. Engaging patients and their families, industry, payers, and other stakeholders in the priority setting enterprise can help ensure that the needs of various stakeholders are met, validating and strengthening the priority setting rationale.

Having a cohesive community to set research priorities is of the upmost importance for rare disease populations, as research resources—including individuals who are eligible to participate, healthcare facilities capable of treating those patients, and financial support for the research—are limited. Rare disease communities of patients, caregivers, and researchers can work together to set research priorities to ensure that the questions that matter the most to patients and their families are answered first.

Below is a graphic representation of PCORI's research topic generation and prioritization process, which may be helpful in thinking of best practices for rare disease research communities to consider in setting a unified research agenda.

Topic Generation and Research Prioritization

1. Receive Topics

Topics can originate from:
i. The Board of Governors
ii. Workshops/Roundtables
iii. Advisory Panels
iv. Engagement with stakeholders
v. Evidence synthesis
vi. Online topic submission form

2. Determine Eligibility

3. Topic Briefs

4. Advisory Panels (APs)

5. Further Prioritization

• Landscape review (as needed)
• Workgroup (as needed)
• Staff recommendation for Targeted PFA, Pragmatic Clinical Studies PFA, or Broad PFA

6. Categorize Opportunity

Targeted PFA
or
Large Pragmatic Studies PFA
or
Special interest in a broad PFA

7. Board of Governors Vote

8. PCORI Funding Announcement (PFA)

27 AP-prioritized topics included to date

Purpose

The purpose of this workgroup is to consider and identify best practices to unify communities, stakeholders, and patients when soliciting research priority questions to set a rare disease research agenda. Questions for this group to consider include:

- o *What are some good examples of cohesive rare disease research communities that were able to come to consensus regarding the research priorities for a given rare condition?*
- o *What are some best practices to engage the patient and stakeholder community in setting a research agenda?*
- o *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?*

Breakout Session Structure

10:30 – 11:15 a.m.: Discuss the questions posed above.

11:15 – 11:45 a.m.: Define a preliminary set of objectives for the workgroup.

11:45 – 12:15 a.m.: Develop a set of next steps and consider what information and resources you need to achieve the objectives you have outlined. Specifically, consider the following:

- Are there specific types of expertise that should be represented on this workgroup that are currently missing? If so, can you recommend someone with that expertise?
- In order to inform future discussions, would a synthesis of the existing literature on this topic be useful? If so, what keywords/MeSH terms should be included in the search?
- Are there other resources that you need to carry out this work?

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 that provides guidance to 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 guidance 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.*

References

1. Cowan, Katherine and Oliver, Sandy. 2013. *The James Lind Alliance Guidebook*. The James Lind Alliance. http://www.jlaguidebook.org/pdfguidebook/guidebook.pdf.
2. Whitlock, Evelyn P., Lopez, Sarah A., Chang, Stephanie, et al. 2008. "Identifying, Selecting, and Refining Topics." In *Methods Guide for Effectiveness and Comparative Effectiveness Reviews*. Rockville, MD: Agency for Healthcare Research and Quality.