



Prioritizing Future Panel Meeting Topics and Initiatives

Range: Best score

1 = Highest

11 = Lowest

Identify and develop cross-cutting ideas for rare disease research, e.g., care coordination, delivery models, research engagement strategies, recruitment and retention strategies, etc.	3
Develop a strategy for soliciting input from rare disease patients, advocacy organizations, researchers, and patient partners	4
Discuss ways for PCORI to assist patient advocacy organizations with developing research proposals for PCORI beyond current resources (e.g., with tailored tools or advice for the rare disease community, besides general grant writing)	4
Set mid- and long-term goals and create annual goals for the RDAP	4

Prioritizing Recommendations for the Future Direction of PCORI Rare Disease Research

1 = Highest

11 = Lowest

Release smaller grants or simplify/streamline application process	3
Develop an application that builds partnerships for research (academic, payer, for profits, government bodies) to build capacity for CER on rare diseases	4
Allow adaptive research designs	4
Sponsor and/or conduct a data mining project to determine types of rare disease research and feasibility for the network(s)	4.5

Prioritizing Suggestions for Internal PCORI Follow-Up

1 = Highest

10 = Lowest

Conduct landscape or systematic review of cross cutting rare disease research to identify opportunities and gaps that PCORI could fund (e.g., diagnostic journey, engagement strategies, patient and caregiver burdens, patient recruitment and retention, care delivery models, etc.)	2
Finalize a core outcome set for pediatrics rare disease initiative with assistance from the RDAP	3.5
Create a workgroup of rare disease researchers from PCORnet, other research networks, PCORI staff, and selected external members (e.g., NIH and VA) to discuss collaboration and prioritizing research questions for the networks (e.g., how to overcome lack of specific diagnostic codes, data platform(s), technology enhancement requirements, etc.)	3
Conduct a workshop at PCORI's annual meeting to train applicants in submitting rare disease research proposals	4

Prioritizing Suggestions for Future Dissemination and Implementation Efforts

4. Please score the below suggested topics for PCORI internal staff to further pursue in order of priority.

1 = Highest

5 = Lowest

Identify targeted and specific dissemination & implementation initiatives early in study development	2
Develop a network during the ongoing study to later use for dissemination & implementation	2.5
Engage with investigators and patients/stakeholders throughout the study to develop key messages for later dissemination & implementation activities	2.5
Identify Ambassadors who are interested in dissemination & implementation of RD research	4
Develop Wikipedia writers to assist with disseminating results of PCORI funded studies	5
