

Advisory Panel on Rare Disease Summer 2019 Webinar

June 12, 2019

3:00 PM – 5:00 PM ET

Dial-in number (US): 1 877 309 2074

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Webinar URL:

<https://attendee.gotowebinar.com/register/6056141230792011011>

Webinar ID: 308-905-923

Housekeeping



- Today's webinar is open to the public and is being recorded
- Members of the public are invited to listen to the teleconference and view the webinar
- Meeting materials can be found on the PCORI website
- Anyone may submit a comment through the webinar chat function, although no public comment period is scheduled
- Visit www.pcori.org/events for more information

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- To queue yourself up to speak, please press the “raise hand” button, or send a message to Allie Rabinowitz using the chat feature.

COI Statement



Welcome to the Rare Disease Advisory Panel in-person meeting. I want to remind everyone that disclosures of conflicts of interest of members of the Advisory Panel are publicly available on PCORI's website. Members of the Rare Disease Advisory Panel are reminded to update your conflict of interest disclosures if the information has changed, in addition to completing your annual disclosure. You can do this by contacting your staff representative, Allie Rabinowitz.

Finally, if the Rare Disease Advisory Panel will deliberate or take action on a matter that presents a conflict of interest for you, please inform one of the co-chairs so we can discuss how to best address the issue.

Roll Call



- Please confirm your attendance when your name is called

Rare Disease Advisory Panel – PCORI Staff



Parag Aggarwal, PhD
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Welcome and Setting the Stage

Matt Cheung, PhD, RPh
RDAP Chair

Cindy Luxhoj, MUP
RDAP Co-Chair

Today's Agenda



Start Time	Item	Speakers
3:00 pm	Welcome and Setting the Stage	M. Cheung / C. Luxhoj
3:15 pm	Prioritizing Future Panel Meeting Topics and Initiatives	M. Cheung
3:50 pm	Prioritizing Recommendations for the Future Direction of PCORI Rare Disease Research	C. Luxhoj
4:25 pm	Blue Cross Blue Shield Training Initiative	N. Aronson
4:40 pm	Closing, Recap, and Future Steps	M. Cheung / C. Luxhoj
5:00 pm	Adjourn	

Survey Results: Prioritizing Future Panel Meeting Topics and Initiatives

Matt Cheung, PhD, RPh, RDAP Panel Chair

Cindy Luxhoj, MUP, RDAP Panel Co-Chair

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Prioritizing Future Panel Meeting Topics and Initiatives (1=Highest)



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

Survey Results: Prioritizing Recommendations for the Future Direction of PCORI Rare Disease Research

Matt Cheung, PhD, RPh, RDAP Panel Chair

Cindy Luxhoj, MUP, RDAP Panel Co-Chair

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Prioritizing Recommendations for the Future Direction of PCORI Rare Disease Research (1=Highest)



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

Blue Cross Blue Shield Training Development

Naomi Aronson, PhD

Executive Director, Clinical Evaluation, Innovation, and Policy,
Blue Cross and Blue Shield Association

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Closing and Next Steps

Matt Cheung, PhD, RPh, RDAP Panel Chair

Cindy Luxhoj, MUP, RDAP Panel Co-Chair

Parag Aggarwal, PhD, Associate Director, Healthcare Delivery and Disparities Research, PCORI

Adjourn

