



Moving Beyond Population Averages: Patient Principles for a Personalized Medicine Research Agenda

Web Forum #2

January 31, 2019

12:00 pm - 1:30 pm EST



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Agenda

I. Welcome and Overview

- Instructions
- PMC Mission and PCORI Project Introduction
- Feedback from Web Forum #1 Participant Survey
- Results from Web Forum #1 Polling Questions
- Guest Speaker Introduction: Mary Jane Marchisotto, Food Allergy Research and Education, and Paul Gross, Cerebral Palsy Research Network

II. Guest Speakers

- Presentations on Patient Involvement in FARE and CPRN PCORI-Funded Projects
- Results of the PCORI-Funded Projects
- Lessons Learned and Best Practices from the PCORI-Funded Projects

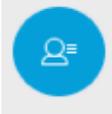
III. Discussion and Next Steps

- Web Forum #2 Polling Questions



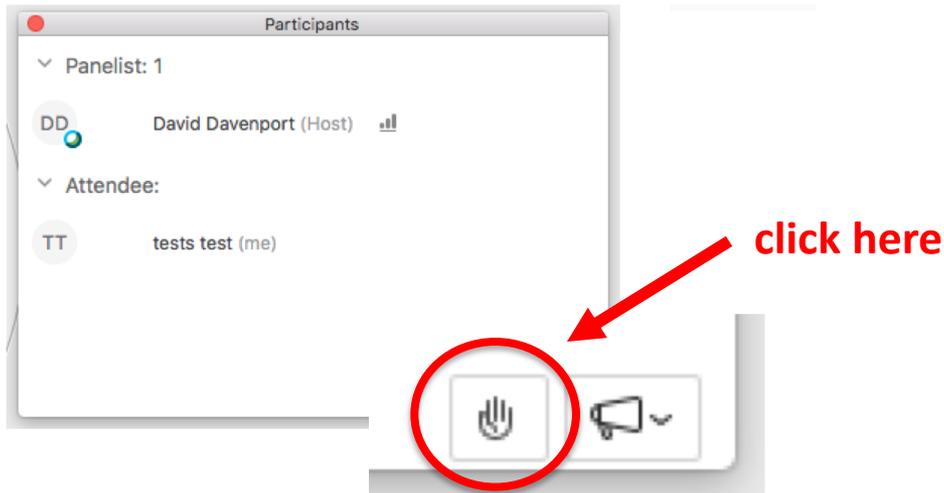
Instructions

- Your audio is muted. You will need to raise your hand to be unmuted. **To raise your hand:**

1. Open the “Participants”  feature.



2. Select the “raise your hand”  button:



Instruction

- Your video webcam will be disabled.
- Q&A and polling features are enabled. These are located in the sidebar on your screen and we will use them during the discussion period.
- The web forum will be recorded. It will be archived for future viewing and dissemination.
- If you need technical assistance, please contact the Host through the Q&A feature, or email ddavenport@personalizedmedicinecoalition.org.

What is Personalized Medicine?

Personalized medicine, often referred to as precision medicine, is an evolving field in which physicians use molecular diagnostic tests to determine which medical treatments will work best for their patients. By combining the data from those tests with an individual's medical history, circumstances, and values, health care providers and patients can develop targeted treatment and prevention plans.



Personalized Medicine Coalition

The Personalized Medicine Coalition (PMC) is comprised of more than 230 innovators, scientists, patients, providers and payers, promotes the understanding and adoption of personalized medicine concepts, services and products to benefit patients and the health system.



Project Funding

This project, *Moving Beyond Population Averages: Patient Principles for a Personalized Medicine Research Agenda*, was selected by the Patient-Centered Outcomes Research Institute (PCORI) to receive a Eugene Washington Engagement Award because it will establish partnerships and build a community equipped to participate as partners in advancing patient-centered clinical effectiveness research.



Project Overview

- We will work with you to develop a set of patient-centered principles and a process to advance personalized medicine during four web forums over the next year.
- We will invite approximately 45 patients, caregivers, researchers and health care providers to an in-person roundtable in Washington, D.C., in 2020 at which participants will translate the principles into a research agenda.
- We will publish the complete research agenda in the form of a white paper in 2020.
- The research agenda will be publicly disseminated to inform future studies that will provide patients, caregivers and clinicians with the evidence they need to make more informed health care decisions.

Web Forum #1 Participant Survey Feedback

- PMC received responses to the survey from about 1/3 of the web forum participants. Thank you!
- Participants found the presentations informative and said they learned something they did not know before the web forum.
- Participants thought the web forum was well organized but would like us to provide materials in advance.
- Participants felt that the tie in with the guest speaker presentation(s) should be made clearer.
- Participants would like more time for Q&A and discussion.

PMC's Response to Participant Survey Feedback

- We will circulate the evaluation survey more quickly after the web forum to increase the number of respondents.
- We will circulate agendas and slides before the remaining web forums.
- We took more time to prepare the speakers in advance of the web forum today and we will tie in specific examples from their presentations during the discussion period that follows.
- We lengthened the web forum overall to allow more time for Q&A and discussion.

Findings from Web Forum #1 Polling Questions

- Participants who responded reported that there was a personalized medicine treatment available for their disease/condition.
- Of those who reported an available personalized medicine treatment, most were not aware of it prior to diagnosis.
- When asked where information was received about personalized treatment options, the primary source identified was a patient advocacy organization.
- Participants reported that it is important for physicians to consider a patient's values and circumstances when recommending a personalized medicine treatment strategy but that few resources were available to facilitate those types of conversations.

Guest Speakers



Mary Jane Marchisotto

SVP Research & International
Food Allergy Research &
Education (FARE)



Paul Gross

Chairman and Founder
Cerebral Palsy Research Network
(CPRN)

Discussion

1. On our last web forum, participants reported that it is important for physicians to consider a patient's values and circumstances when recommending a personalized medicine treatment strategy. Participants noted that the research agenda would be most helpful if it focused on priorities that help providers in:

- A. Identifying and communicating treatment options based biological differences in the context of **patient/caregiver values**
- B. Understanding **patient/caregiver circumstances** and presenting treatment options in ways that highlight how a treatment aligns/does not align with those circumstances

Should research guiding health care provider communication be a primary focus of PMC's research agenda? **Yes or No.** Who else should we consider as a focus for the research agenda? **DISCUSS**

Discussion (Continued)

2. For our last web forum, we defined **patient/caregiver values** to be personal priorities, religious/spiritual values, societal and cultural values (including family involvement in care decisions), views around quality of life, and beliefs about health and personal responsibility. Do you agree that this is an accurate definition of **patient/caregiver values**? **Yes or No**. If no, what other factors should we consider including? **DISCUSS**

3. For our last web forum, we defined **patient/caregiver circumstances** to be emotional state, economic situation, access to care, social support, cognitive abilities, attitude toward illness, relationship with the healthcare provider, and role of patient as a caretaker. Do you agree that this is an accurate definition **patient/caregiver circumstances**? **Yes or No**. If no, what other factors should we consider including? **DISCUSS**

Discussion (Continued)

4. On today's web forum, you heard from FARE about the organization's project to establish a research agenda for food allergy. FARE used a structured process for soliciting applications and selecting members for their project advisory committee. Should PMC follow a similar process soliciting applications and selecting members of our advisory committee? **Yes or No.**

5. What stakeholder groups should we include in our 45 person advisory committee to ensure that we focus our research agenda on priorities that fill gaps in treatment decision making and communication aligned with patient/caregiver values and circumstances?

- A. Patients and caregivers
- B. Patient advocacy organization representatives
- C. Academic researchers
- D. Health care providers
- E. Payers
- F. Pharmaceutical and diagnostic industry representatives
- G. Federal partners (National Institutes of Health, Food and Drug Administration, Centers for Medicare and Medicaid, etc.)
- H. Other

Discussion (Continued)

6. During today's web forum you heard from the CPRN about the organization's project to establish a research agenda for cerebral palsy. The CPRN used an online voting system to build consensus around priority research areas to pursue through their research network. Given that there is interest in involving other stakeholders in the agenda setting process, should PMC consider incorporating a similar consensus building mechanism to further vet the principles we are developing together through these web forums? **YES or NO.**

7. Are there other elements of the FARE and CPRN projects that that PMC should adopt as part of the process to set our research agenda? **DISCUSS**

Next Steps

- You will receive an evaluation survey for this web forum. We would encourage you to fill it out so that we can see how we did and how we can improve.
- Post web forum archive and slides. These will be available on PMC's website, www.personalizedmedicinecoalition.org
- We will take feedback from this program and use it to inform our next web forum. Target timeframe is late May-early June 2019.
- Continue outreach. If there are individuals or organizations you feel would benefit from this program, please let us know.
- If you have an idea, stay in touch!

Thank you for your attention.
Questions or comments, please contact us.

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