Moving Beyond Population Averages: A Roundtable to Develop a Patient-Centered Research Agenda Advancing Personalized Medicine

Session 1: Patient-Centered Principles for a Personalized Medicine Research Agenda

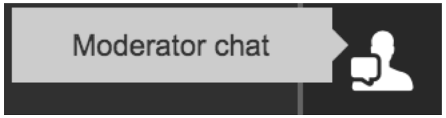
June 19, 2020
1:00 – 3:00 p.m. ET

PMC
PERSONALIZED
MEDICINE COALITION

Welcome!

We will start today's session momentarily.

Please turn on your video camera and mute your microphone.



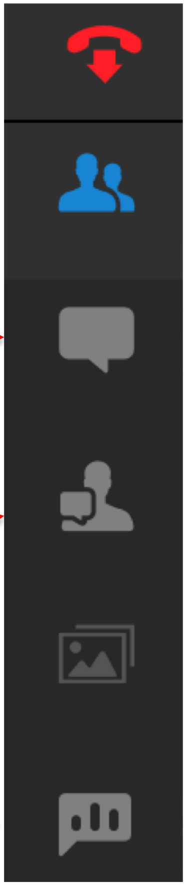
If you need technical assistance, please contact our support team using the "moderator chat" icon on the right side of your screen.

PMC
PERSONALIZED
MEDICINE COALITION

Note: This videoconference will be recorded and archived for future viewing and public dissemination.

Using Videoconference Features

- Keep your microphones **muted** to minimize interruptions.
- During the panel discussions, please ask questions through the **event chat feature**.
- If you need technical assistance, please contact support through the **moderator chat icon**.
- To open a polling question, select the **polls icon**, then click and submit your response.
- To adjust the size of the video or the slides for better viewing, hover over them and use the **video/slide slider bar** that appears at the bottom.



PMC
PERSONALIZED MEDICINE COALITION

Moving Beyond Population Averages: A Roundtable to Develop a Patient-Centered Research Agenda Advancing Personalized Medicine

Session 1: Patient-Centered Principles for a Personalized Medicine Research Agenda

June 19, 2020
1:00 – 3:00 p.m. ET

PMC
PERSONALIZED
MEDICINE COALITION

Who We Are

Cynthia A. Bens

Senior Vice President,
Public Policy

Personalized Medicine Coalition

Engagement Award Project Lead

Daryl Pritchard, Ph.D.

Senior Vice President,
Science Policy

Personalized Medicine Coalition

Engagement Award Scientific Content Officer

David Davenport

Manager, Public Policy

Personalized Medicine Coalition

Engagement Award Project Manager

Faswilla Sampson, M.S.

Vice President, Operations

Personalized Medicine Coalition

Engagement Award Project Administrator

PMC
PERSONALIZED
MEDICINE COALITION

Session #1 Agenda

I. Welcome and Introduction
  - What is Personalized Medicine?
  - PCORI Engagement Award Project Overview
  - Roundtable Objectives & Participants
  - Ground Rules for Discussion

II. Panel Discussion: Patient-Centered Principles for a Personalized Medicine Research Agenda

III. Group Discussion: Framing Research Questions in the Context of the Patient-Centered Principles to Improve the Delivery of Personalized Medicine

PMC
PERSONALIZED MEDICINE COALITION

Session #1 Agenda (cont'd)

IV. Wrap-Up and Next Steps

- Summary of Key Takeaways
- Sharing Additional Feedback
- Evaluation Survey

V. Conclusion

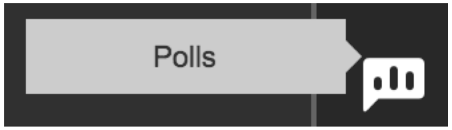
PMC
PERSONALIZED
MEDICINE COALITION

Polling Question #1

How would you rate your understanding of personalized medicine?

A. I have heard of it, but do not understand what it is.
B. I understand what personalized medicine is.
C. I or someone I know has been treated with personalized medicine.
D. I advocate for personalized medicine or use it in my profession.

To open a polling question, select the ***polls icon.***



PMC
PERSONALIZED
MEDICINE COALITION

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.

PMC
PERSONALIZED
MEDICINE COALITION

What is Patient-Centered Outcomes Research?

Patient-centered outcomes research helps people and their caregivers communicate and make informed healthcare decisions, allowing their voices to be heard in assessing the value of healthcare options. This research answers patient-centered questions, such as:

- “Given my personal characteristics, conditions, and preferences, what should I expect will happen to me?”
- “What are my options, and what are the potential benefits and harms of those options?”
- “What can I do to improve the outcomes that are most important to me?”
- “How can clinicians and the care delivery systems they work in help me make the best decisions about my health and health care?”

Source: https://www.pcori.org/research-results/patient-centered-outcomes-research

PMC
PERSONALIZED
MEDICINE COALITION

Why a Patient-Centered Research Agenda for Personalized Medicine?

- There are fundamental gaps in awareness and evidence that impact the speed at which personalized medicine products and services are integrated into health care.

- Personalized medicine and patient-centered outcomes research (PCOR) share the same goal of shifting healthcare from a one-size-fits-all, trial-and-error approach toward a targeted approach that utilizes patients' characteristics and preferences to inform healthcare decisions.

- PMC is engaging stakeholders across the healthcare spectrum to develop a research agenda that is informed by early successes in personalized medicine adoption and builds on principles defined by patients.

Project Overview

- Over the past year, PMC has been convening patients, patient advocates and caregivers, along with other stakeholders, to develop a set of patient-centered principles for the research agenda.

- PMC formed a multi-stakeholder advisory committee to help translate those principles into impactful research questions and plan this meeting.

- This series of virtual roundtable discussions will help refine research questions for the agenda by bringing together a diversity of perspectives.

- In August, we will publish a white paper with the final research agenda and a summary of the roundtable discussions.

- PMC will disseminate the research agenda to inform future studies that will provide patients, caregivers and clinicians with the information they need to make more informed healthcare decisions.

Project Funding

This project 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.

Patient-Centered Principles

The patient community has identified nine priorities for the research agenda. These cover the following themes:

- Defining patient/caregiver values and circumstances
- Improving patient-provider communication
- Improving the education of patients and providers
- Access challenges (e.g., coverage, affordability, and provider availability)
- Representativeness (e.g., diverse patient needs, diseases, backgrounds, and experiences)
- Impact and feasibility

PMC
PERSONALIZED
MEDICINE COALITION

Roundtable Objectives

1. To refine language in the proposed research questions to be most impactful and relevant to the patient priorities identified in the patient-centered principles.

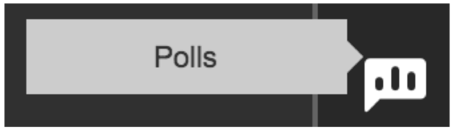
2. To discuss suggestions for additional research questions that you think would improve patients’ outcomes and experiences with personalized medicine.

Polling Question #2

Which of the following best describes you?

A. Patient/Advocate/Caregiver
B. Patient Advocacy Organization Representative
C. Health Care Professional/Provider
D. Academic Researcher/Outcomes Researcher
E. Other

To open a polling question, select the ***polls icon.***



PMC
PERSONALIZED
MEDICINE COALITION

Roundtable Participants

Disease Areas

- Alzheimer’s
- Cancer (breast, lung, multiple myeloma, colorectal, lymphoma, rare blood, genetic risk)
- Caregiving
- COPD
- Cystic fibrosis
- Food allergy
- Health disparities
- HIV
- Kidney disease
- Rare diseases

Areas of Expertise

- Community health
- Diagnostics
- Family medicine
- Genetic counseling
- Genomics education
- Health insurance
- Health IT
- Hospital and program administration
- Nursing and nurse education
- Outcomes research
- Pharmacogenomics
- Practicing physicians/researchers

PMC
PERSONALIZED MEDICINE COALITION

Ground Rules for Discussion

1. Introduce yourself when you speak.
2. Be respectful.
3. Share the mic, but don’t be afraid to speak up when you have something to say.
4. Ask for clarification if you are confused.
5. Speak from your own experience whenever possible.
6. Critique ideas, not people.
7. Build on one another’s comments; work toward a shared understanding.

PMC
PERSONALIZED
MEDICINE COALITION

Ground Rules for Discussion (cont'd)

8. Recognize that each participant has a different perspective, experience, and area of expertise; be willing to learn from each other.

9. Consider and be willing to address the diversity of patient needs, diseases, backgrounds, and experiences.

10. Follow up with us afterward if you were not able to share a comment you think is important for us to consider.

11. Understand that even though we do not have the answer to a question, you should still ask it. We want to identify those unanswered questions.

PMC
PERSONALIZED
MEDICINE COALITION

Moderator

Susan McClure

Founder, CEO, Genome Creative

Panel Discussion: Patient-Centered Principles for a Personalized Medicine Research Agenda

Moderator | Susan McClure, Founder, CEO, Genome Creative

Laura Holmes Haddad, Writer

Gabriella Balasa, Patient, Advocate, Cystic Fibrosis

C. Grace Whiting, J.D., President, CEO, National Alliance for Caregiving

Adolph P. Falcón, M.P.P., Executive Vice President, National Alliance for Hispanic Health

Please submit questions for speakers through the event chat.

PMC
PERSONALIZED
MEDICINE COALITION

Patient-Centered Principles

1. **Patient/caregiver values** include personal priorities, religious/spiritual values, societal and cultural values (including family involvement in care decisions), views around quality of life, privacy concerns, desired level of access to and understanding of personal genetic information, beliefs about health and personal responsibility, and attitudes and preferences toward end of life.

2. **Patient/caregiver circumstances** include emotional state, socioeconomic situation, race/ethnicity, sex/gender, language, health literacy, ability to work, access to care, access or lack thereof to a caregiver, social support, cognitive abilities, attitude toward illness (e.g., acceptance of diagnosis, willingness to accept help), personality, symptom burden, health-related quality of life, ability to consent and choose, relationship with the health care provider, the role of patient as a caretaker, preferences of family members, treatment setting (e.g., community, academic, other), familiarity with personalized medicine and its benefits, other social determinants of health, and other expressed needs or barriers.

PMC
PERSONALIZED
MEDICINE COALITION

Patient-Centered Principles

3. The research agenda would be most helpful if it focused on priorities that help providers in (a) **identifying and communicating treatment options** based on biological differences in the context of patient/caregiver values and (b) **understanding patient/caregiver circumstances** and presenting treatment options in ways that highlight how a treatment aligns/does not align with those circumstances.
    - The potential for shared decision-making tools and resources to facilitate these conversations should be explored.

(continued)

PMC
PERSONALIZED
MEDICINE COALITION

Patient-Centered Principles

4. The research agenda should address the **education of patients** directly by considering (a) the education of patients/caregivers as a continuing process over time and not just at diagnosis, including basic education defining personalized medicine and its benefits; (b) how both patients/caregivers and their providers can improve communication with each other; and (c) how to close the gap between patient/caregiver expectations and the reality of how/when test results and treatment options are delivered.
   - Educational tools and resources, including those available online, should address different learning styles and levels of health literacy.

Patient-Centered Principles

5. Opportunities to **educate providers** in genomics should be considered, along with providers' varying levels of understanding of genomics and varying levels of access to new and existing resources, such as translational tools, depending on their health setting (e.g., community, academic, other), health specialization, and health discipline (e.g., pharmacists, physician assistants, genetic counselors, nurses).

    - This includes opportunities to educate providers and other health care professionals in training.

6. The agenda should consider how to use **oncology as a prototype** for patient and provider education in disease areas beyond oncology.

Patient-Centered Principles

7. **Access challenges** beyond those created by a lack of communication and education should also be considered.
   - This includes timely access to novel and/or off-label therapies as a result of affordability, insurance coverage and provider availability.

8. The research agenda should be **representative** of diverse patient needs, diseases, backgrounds, and experiences (e.g., stage of life, disease trajectory, socioeconomic status and health literacy level), including patients underrepresented in medical research.
   - Specifically, the research agenda should apply across disease areas.
   - This includes considering how the research agenda can apply to additional disease areas as new personalized medicine treatments come to market.
   - This includes potential research topics related to the management of comorbidities.

PMC
PERSONALIZED
MEDICINE COALITION

Patient-Centered Principles

9. The advisory committee should consider areas the agenda must include and exclude to have the greatest **impact**, including the **feasibility** of proposed research topics.
    - This includes potential research topics related to artificial intelligence, data collection, data integration and interoperability, informed consent, and patient concerns related to data privacy and access.

PMC
PERSONALIZED
MEDICINE COALITION

Proposed Research Questions

1. How can education, communication and healthcare delivery strategies close the existing gap between a **patient/caregiver's expectations** about personalized medicine and the **reality** of how and when test results and treatment options are currently delivered?

2. What kinds of new and existing **educational tools and resources**, including those using digital platforms, would empower patients and providers, with varying levels of understanding about personalized medicine and its benefits, to discuss personalized medicine treatment options during visits?

3. How can patients and caregivers be encouraged to more **actively participate** in their personalized treatment when they prefer not to think about it, want to rely on their trusted medical team, or feel inadequate to participate?

PMC
PERSONALIZED
MEDICINE COALITION

Proposed Research Questions (cont’d)

4. How can conversations between a patient and health care professional about personalized medicine treatment options best address issues related to **access and affordability** of care?

5. How can healthcare professionals and patients navigate **language and cultural differences** when discussing personalized medicine treatment options or delivering/receiving care?

6. How do a patient’s needs and access barriers, including those related to cost and affordability, change depending on the **purpose of a genetic test** (e.g., to identify risk of developing a disease vs. to identify an appropriate treatment)?

7. How can outcomes research in personalized medicine consider the impact of treatment on a **patient’s financial health, psychosocial well-being, and experience receiving care**?

PMC
PERSONALIZED
MEDICINE COALITION

Group Discussion: Framing Research Questions in the Context of the Patient-Centered Principles to Improve Delivery of Personalized Medicine

Moderated by
Susan McClure
Founder, CEO, Genome Creative

Please remember to mute your line when you are not speaking.

PMC
PERSONALIZED
MEDICINE COALITION

Summary of Key Takeaways

PMC
PERSONALIZED
MEDICINE COALITION

Next Steps

- If you have additional comments or questions, please email them to David Davenport at ddavenport@personalizedmedicinecoalition.org.
- We will take your feedback and incorporate it into the final research agenda, which will be published in the form of a white paper in August 2020.
- We will share the session recording and slides and post them on PMC's website: http://www.personalizedmedicinecoalition.org.
- You will receive an evaluation survey for this session. We encourage you to fill it out so that we can see how we did and how we can improve future sessions.
- Please join us for our next session on Monday, June 22, which will focus on "Practical Considerations to Improve the Delivery of Personalized Medicine within the Current Health Care System."

PMC
PERSONALIZED
MEDICINE COALITION

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

David Davenport
ddavenport@personalizedmedicinecoalition.org
804-291-8572

Cynthia A. Bens
cbens@personalizedmedicinecoalition.org
202-499-0986

www.personalizedmedicinecoalition.org
Twitter: @permedcoalition

PMC
PERSONALIZED
MEDICINE COALITION