Evidence and the Individual Patient: Understanding Heterogeneous Treatment Effects for Patient-Centered Care

MAY 31, 2018
NATIONAL ACADEMY OF SCIENCES BUILDING
LECTURE ROOM
2101 CONSTITUTION AVE NW
WASHINGTON, DC 20418

Meeting Focus: Leveraging data to examine heterogeneous treatment effects to personalize and improve patient care

Motivating Questions:
1. Potential: How can clinical trial data be analyzed to yield reliable patient-centered treatment effect estimates? What are the state-of-the-science methods for assessing treatment heterogeneity?
2. Risks: How can we be sure personalizing evidence will improve decision making, as compared to the default of relying on overall average treatment effects? What are the evidentiary standards for implementing changes to clinical practice to personalize care based on evidence of heterogeneous treatment effects?
3. Lessons Learned: What can be learned from the challenges of genomics-based personalized medicine? What can be learned from the efforts of previous clinical trialists to understand more personalized treatment effect estimates?
4. Strategies: How should clinical research and clinical practice be redesigned to support the generation and dissemination of patient-centered evidence?

Outcomes Anticipated: The conference will stimulate discussion and further collaborative action to advance the research and policy agenda for patient-centered evidence, and will inform the development of a white paper outlining the optimal methodological approaches to personalizing treatment effects, and the clinical contexts in which these approaches are likely to be of most value.

8:30 am Coffee and light breakfast available

9:00 am Welcome, introductions, and meeting overview
Welcome from the National Academy of Medicine
Anne-Marie Mazza, National Academy of Medicine
Opening remarks and meeting overview
Joe Selby, Patient-Centered Outcomes Research Institute
9:15 am  **Overview of heterogeneous treatment effects: Moving from evidence-based medicine to personalized/precision medicine**

Speakers will present a conceptual overview of heterogeneous treatment effects, as well as examples of clinical trials analyzed to yield more personalized treatment effect estimates. Discussion will focus on how changes in the design of clinical research might enable a better understanding of how treatment effects vary across individuals.

Moderator: *Harry Selker*, Tufts Medical Center

Speakers:
*David Kent*, Tufts Medical Center
*Sanjay Basu*, Stanford University
*Derek Angus*, University of Pittsburgh

Discussants:
*Sheldon Greenfield*, University of California at Irvine
*Bob Temple*, FDA

**Q&A and Open Discussion**

10:45 am  **Break**

11:00 am  **An equation-free presentation of new methods for prediction of treatment benefit and model evaluation**

This session will focus on statistical methods. Speakers will discuss lessons learned from the genomics revolution, machine learning methods for the analysis of trial data, and new methods for evaluating models that predict treatment benefit.

Moderator: *Ewout Steyerberg*, Leiden University Medical Center;

Speakers:
*A. Cecile Janssens*, Emory University Rollins School of Public Health
*Fan Li*, Duke University
*Patrick Heagerty*, University of Washington School of Public Health

Discussants:
*Frank Harrell*, Vanderbilt University
*Michael Pencina*, Duke Clinical Research Institute

**Q&A and Open Discussion**
12:20 pm  **Break**  
Participants will pick up lunch.

12:35 pm  **Discussion with stakeholders**  
This session will focus on how representatives of several patient communities have applied research to guide their own care, given their own individual circumstances. Additional stakeholders will contribute to the discussion of how to better align evidence with patient-centered care.

Moderator:  *Bray Patrick-Lake*, Duke University

Panelists:  
*Thomas Concannon*, RAND Corporation  
*Seth Morgan*, National Multiple Sclerosis Society Advocate, Patient Stakeholder  
*Christine Stake*, Lurie Children's Hospital, Patient Stakeholder

Reactors:  
*Robert Dubois*, National Pharmaceutical Council  
*Karina Davidson*, Columbia University

*Q&A and Open Discussion – engagement with other stakeholders*

1:30 pm  **Break**

1:45 pm  **From Research into Practice: Implementation and oversight**  
This session will focus on barriers to implementation applying predictions in clinical practice and how to overcome these barriers. Speakers will discuss how to go beyond “all-or-nothing” quality measures to incentivize more personalized care.

Moderator:  *Nilay Shah*, Mayo Clinic

Speakers:  
*John Spertus*, Saint Luke's Mid America Heart Institute  
*Josh Peterson*, Vanderbilt University  
*Rodney Hayward*, University of Michigan

Discussants:  
*Naomi Aronson*, Blue Cross Blue Shield Association  
*Katrina Armstrong*, Massachusetts General Hospital

*Q&A and Open Discussion*

3:15 pm  **Opportunities for collaborative action**  
The aim of this session is to reflect on key themes from the day’s discussion, focusing on innovative methods for understanding heterogeneous treatment...
effects, challenges related to implementation and oversight to personalize care, and outstanding policy and research questions that need to be addressed to accelerate progress.

Moderator: Joe Selby, PCORI
Reactors: Steven Goodman, Stanford University; Evelyn Whitlock, PCORI

Closing Remarks: David Kent, Tufts Medical Center

| 4:30 pm | Adjourn |

Planning Committee

Thomas Concannon, PhD, MA, RAND Corporation
Robert Golub, MD, Journal of the American Medical Association
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