



## Virtual Clinical Trials: Challenges and Opportunities

November 28 – 29, 2018

National Academy of Sciences Building, Lecture Room  
2101 Constitution Ave. NW, Washington, DC 20418

**Background:** The cost of bringing a novel drug to market is now estimated to exceed 2.5 billion dollars and over 90% of drug candidates fail in late-stage, phase 3 clinical trials. Some argue that these challenges are due in part to outmoded and inefficient clinical trial platforms. Technological advances have opened the possibility for new trial designs that could increase participation in the United States, particularly through engagement of community health centers or other local providers beyond the traditional academic center. Digital health technologies and virtual trial designs (e.g., trials that deploy various digital health tools or remote site visits) could create more equitable and representative recruitment of participants as well as for more effective uptake of meaningful, generalizable trial findings into clinical practice.

Since 2009, the Forum on Drug Discovery, Development, and Translation of the National Academies of Sciences, Engineering, and Medicine has been engaged in a focused effort, anchored by a multi-workshop series, to address challenges facing the U.S. clinical trials enterprise and to engage stakeholders in an ongoing discussion of potentially transformative strategies to improve the efficiency and effectiveness of clinical trials. This workshop will build upon this work and examine opportunities for a modern clinical trials enterprise in light of other transformative changes in the drug development and health system sectors.

**Workshop Objectives:** This workshop will examine opportunities for a modern, patient-centric clinical trials enterprise in light of digital health tools that could allow a virtual clinical trial for new medical product approval. Subject matter experts will engage in presentations and discussions to:

- Highlight opportunities for systemic improvements to support virtual clinical trials, including:
  - potential implications of virtual clinical trials for cost, speed, regulation, and knowledge generation and dissemination; and
  - elements of an IT infrastructure, including integrating data from EHRs, mobile health applications, remote monitoring, virtual visits, and other relevant technologies with the capability to enhance the interface between clinicians and clinical trial participants.
- Explore potential opportunities to use digital health tools to engage with patients and potential research participants, facilitate recruitment of participants to join a clinical trial, and maintain participation of diverse populations in the trial, including:
  - collaborative approaches and incentives involving sponsors, researchers, patient advocacy groups, patients living with the particular condition being studied, and health systems – including regulations, quality measures and outcomes, or reimbursement strategies – to support the implementation of virtual clinical trials; and
  - opportunities and challenges to enhancing equity in access and participation through virtual clinical trials.

# DAY 1: November 28

1:00pm **Welcome and Opening Remarks**

**LINDA BRADY**, *Workshop Co-Chair*

National Institute of Mental Health, National Institutes of Health

**CLAY JOHNSTON**, *Workshop Co-Chair*

University of Texas, Austin

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## SESSION I OPPORTUNITIES TO IMPROVE CLINICAL TRIALS

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### Session Objectives:

- Consider the efficiency and effectiveness of the current clinical trials landscape in the United States – what is working and not working well?
- How could virtual clinical trials improve traditional Phase 3 clinical trials and overall medical product development?

### Session Co-Chairs:

*Linda Brady, National Institutes of Health*

*Clay Johnston, University of Texas, Austin*

1:10pm **RAY DORSEY**

Professor of Neurology and Director, Center for Health and Technology  
University of Rochester Medical Center

**DONNA CRYER**

President and CEO  
Global Liver Institute

**CRAIG LIPSET**

Head of Clinical Innovation, R&D  
Pfizer, Inc.

1:50pm **Discussion with workshop participants**

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## SESSION II EXPLORING VIRTUAL CLINICAL TRIALS

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### Session Objectives:

- Hear a variety of perspectives and experiences with virtual and digital health technologies in interventional and observational studies, as well as clinical care, and highlight opportunities to use these technologies to improve clinical trials of investigational products.
- Discuss challenges and best practices for designing and implementing a virtual clinical trial.

Session Co-Chair:

*Kelly Simcox, Sanofi*

2:15pm      **Lessons Learned from Clinical Care**

**JENNA BOLLYKY**

VP Clinical Research and Analytics  
Livongo Health

2:30pm      **Lessons Learned from Observational Studies**

**JOSHUA DENNY**

Professor of Biomedical Informatics and Medicine  
Vanderbilt University

2:45pm      **Discussion with workshop participants**

3:00pm      **BREAK**

3:15pm      **Lessons Learned from Interventional Virtual Clinical Trials**

**STEVEN CUMMINGS**

Director, San Francisco Coordinating Center  
Professor of Medicine, Epidemiology, and Biostatistics  
University of California, San Francisco

**WENDY WEBER**

Acting Deputy Director  
National Center for Complementary and Integrative Health, National Institutes of Health

**KIMBERLY HAWKINS**

Clinical Sciences and Operations Project Leader Head  
Sanofi Genzyme

4:00pm      **Panel Discussion and Reactions**

**NOAH CRAFT**

CEO  
Science 37

**ADRIAN HERNANDEZ**

Vice Dean for Clinical Research, Duke University School of Medicine  
Faculty Associate Director, Duke Clinical Research Institute

**JON WHITE**

Deputy National Coordinator for Health Information Technology  
Office of the National Coordinator for Health Information Technology  
U.S. Department of Health and Human Services

**JOSH ROSE**  
Vice President, Global Head of Strategy  
IQVIA

4:30pm **Discussion with workshop participants**

5:00pm **Adjourn Day One**

## **DAY 2: November 29**

8:30am **Breakfast**

8:45am **Welcome and Recap Day 1**

**LINDA BRADY**, *Workshop Co-Chair*  
National Institute of Mental Health, National Institutes of Health

**CLAY JOHNSTON**, *Workshop Co-Chair*  
University of Texas, Austin

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### **SESSION III ACCESS AND EQUITY**

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#### Session Objectives:

- Consider how to frame issues of access and equity in the context of virtual trials. Could virtual trials potentially exacerbate current inequities or make access to clinical trials worse for some communities?
- Discuss the potential benefits and risks of end-to-end virtual clinical trials for traditionally underrepresented populations in research.

#### Session Co-Chairs:

*Kathy Hudson, Patient-Centered Research Foundation*

*Rebecca Pentz, Emory University School of Medicine*

9:00am **WILL MCINTYRE**  
Patient Advocate  
Michael J. Fox Foundation for Parkinson's Research

**SALLY OKUN**  
Vice President, Policy and Ethics  
PatientsLikeMe

**SILAS BUCHANAN**  
Chief Executive Officer  
Institute for eHealth Equity

**SHERINE EL-TOUKHY**  
Postdoctoral Research Associate  
National Institute on Minority Health and Health Disparities  
National Institutes of Health

10:00am Discussion with workshop participants

10:30am BREAK

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## SESSION IV POLICY CONSIDERATIONS

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### Session Objectives:

- Discuss existing, and yet to be conceived, policies and standards governing virtual clinical trials for medical product development.
- What are the challenges and potential solutions surrounding the collection of remote data from participants, including how to ensure the data collected is coming from the person you think it is, and how to know they are using the device correctly – all while protecting privacy?
- Consider the landscape of standards and any gaps that may need to be addressed in order to conduct increasingly virtual trials.

### Session Co-Chairs:

*John Wilbanks, Sage Bionetworks*

*David McCallie, Cerner Corporation*

10:45am **LEONARD SACKS**  
Associate Director for Clinical Methodology  
Office of Medical Policy, Center for Drug Evaluation and Research  
U.S. Food and Drug Administration

**LEANNE MADRE**  
Director of Strategy  
Clinical Trials Transformation Initiative

**DEVEN MCGRAW**  
General Counsel and Chief Regulatory Officer  
Citizen Corporation

**MATTHEW MCINTYRE**  
Senior Scientist, Data Collection  
23andMe

11:45am Discussion with workshop participants

12:30pm Lunch

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## SESSION V POTENTIAL FUTURE DIRECTIONS

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### Session Objective:

- Discuss key highlights from the workshop presentations and discussions, including identifying potential next steps and promising areas for future action.

### Session Co-Chairs:

*Linda Brady, National Institutes of Health*

*Clay Johnston, University of Texas, Austin*

### 1:15pm **Observations from the Workshop and Potential Future Directions**

- **Linda Brady and Clay Johnston**, Session I: Opportunities to Improve Clinical Trials
- **Kelly Simcox**, Session II: Exploring Virtual Clinical Trials
- **Kathy Hudson and Rebecca Pentz**, Session III: Access and Equity
- **John Wilbanks and David McCallie**, Session IV: Policy Environment

2:15pm Discussion with workshop participants

3:00pm Workshop Adjourns