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.
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

SESSION I  OPPORTUNITIES TO IMPROVE CLINICAL TRIALS

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 and digital health tools improve traditional Phase 3 clinical trials and overall medical product development?

Session Co-Chairs: Linda Brady and Clay Johnston

1:15pm  RAY DORSEY (confirmed)
Professor of Neurology and Director, Center for Health and Technology
University of Rochester Medical Center

DONNA CRYER (invited)
President and CEO
Global Liver Institute

CRAIG LIPSET (confirmed)
Head of Clinical Innovation, R&D
Pfizer, Inc.

1:45pm Discussion with workshop participants

SESSION II  THE POTENTIAL USE OF DIGITAL HEALTH TOOLS IN CLINICAL TRIALS

Session Objectives:
● Hear from a variety of organizations about 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 studies of investigational products.
● Discuss challenges and best practices for using digital tools to find the right participants for a clinical trial, as well as conducting informed consent remotely.

Session Co-Chairs: Kelly Simcox and Todd Sherer

Experiences with Digital Health Technologies
Lessons Learned: Digital Health Technologies in Clinical Care Settings

[Speaker(s) TBA]

Lessons Learned: Digital Health Technologies in Observational Studies

Joshua Denny (confirmed)
Professor of Biomedical Informatics and Medicine
Vanderbilt University

Learnings from Interventional Clinical Trials Using Digital Health Technologies

Steven Cummings (confirmed)
Director, San Francisco Coordinating Center
Professor of Medicine, Epidemiology, and Biostatistics
University of California, San Francisco

Kimberly Hawkins (confirmed)
CSO Project Leader Head
Sanofi Genzyme

[Additional Speaker TBA]

Panel Discussion and Reactions

Christine Pierre (invited)
President
Society for Clinical Research Sites

Jon White (invited)
Deputy National Coordinator for Health Information Technology
Office of the National Coordinator for Health Information Technology
U.S. Department of Health and Human Services

[Additional Panelists TBA]

Discussion with workshop participants

Adjourn Day One
DAY 2: November 29

SESSION III  ACCESS AND EQUITY

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 and Rebecca Pentz

9:00am  WILL MCINTYRE (*confirmed*)
Patient Advocate

ELISEO PEREZ-STABLE (*confirmed*)
Director, National Institute on Minority Health and Health Disparities
National Institutes of Health

SALLY OKUN (*confirmed*)
Vice President, Policy and Ethics
PatientsLikeMe

[ADDITIONAL SPEAKER TBA]

10:00am  Discussion with workshop participants

10:30am  BREAK

SESSION IV  POLICY ENVIRONMENT

Session Objectives:
- Discuss existing, and yet to be conceived, policies and standards governing the use of digital health tools in 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 and David McCallie
SESSION V  POTENTIAL FUTURE DIRECTIONS

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 and Clay Johnston

1:30pm  Observations from the Workshop and Potential Future Directions

● Linda Brady and Clay Johnston, Session I: Opportunities to Improve Clinical Trials
● Kelly Simcox and Todd Sherer, Session II: The Potential Use of Digital Health Tools in 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