Obtaining Novel Endpoint Reliability and Acceptance

Expert Meeting Agenda

Tuesday, July 27 & Wednesday, July 28, 2021

10:30 a.m. – 1:30 p.m.

Virtual Event

CTTI MISSION: To develop and drive adoption of practices that will increase the quality and efficiency of clinical trials

MEETING OBJECTIVES:

- Identify the barriers and discuss solutions to the adoption of DHT-derived endpoints into pivotal trials
- Discuss the data needed to prove that a DHT-derived endpoint is ready for a pivotal trial
- Explore how collaboration and other new efforts can advance the adoption of DHT-derived endpoints

MEETING SCOPE:

This meeting will center on endpoints related to functional measures and/or other clinical outcome assessments (COAs) that use digital health technologies (DHTs) for data capture.

TUESDAY, JULY 27, 2021

10:30 a.m. Welcome & Opening Remarks

10:30 a.m. Introduction to the Clinical Trials Transformation Initiative (CTTI)
Leanne Madre, CTTI

10:35 a.m. The Value of Digital Health Technology (DHT)-Derived Endpoints
Matthew Diamond, FDA/CDRH
Phil Green, Patient Advocate

10:55 a.m. Session I: DHT-Derived Novel Endpoint Development: Current Landscape

Session Objectives:
- Review existing frameworks for DHT-derived endpoint development
- Highlight the language that will be used during the meeting

10:55 a.m. Framing the Fit for Purpose DHT-Derived COA Endpoint
Elektra Papadopoulos, FDA/CDER

11:05 a.m. Landscape Overview: Resources to Support Novel Endpoint Development
Lindsay Kehoe, CTTI

11:15 a.m. Barriers to and Recommendations for Adoption of DHT-derived Endpoints Review of CTTI's Current Novel Endpoint Project Results
Brian Perry, CTTI

11:35 a.m. Break

11:40 a.m. Session II: DHT-Derived Endpoints in Trials: The Reality vs. the Ideal

Session Facilitator/Moderator: Megan Doyle, Amgen
Session Objectives:
- Discuss barriers to the adoption of DHT-derived endpoints as key endpoints in pivotal trials
- Explore solutions to the adoption barriers
- Share experiences and perspectives of applying existing frameworks for DHT-derived endpoint development

11:45 a.m. Panel Discussion: Stakeholder Perspectives on the Barriers to Adoption of DHT-Derived Endpoints

Beth Kunkoski, FDA/CDER
Cindy Geoghegan, Patient and Partners
Leslie Harden, BIO
Jeremy Wyatt, ActiGraph
Jordan Silberman, Anthem, Inc.

12:25 p.m. Q&A of Panel

12:40 p.m. Break Out Groups
- Explore Solutions to Identified Adoption Barriers

- Potential questions include:
  - Rank the top 3 barriers that are impeding the adoption of DHT-derived endpoints into pivotal trials
  - In an ideal world, what is needed to fix those barriers? (i.e. what needs to change)
  - What would be CTTI's role to help address those barriers?

1:20 p.m. Summary of Break Out Groups
Break Out Group Facilitators

1:25 p.m. Burning Question

1:30 p.m. Adjourn

WEDNESDAY, JULY 28, 2021

10:30 a.m. Welcome & Opening Remarks

Recap of Day 1 and Introduction to Day 2
Lindsay Kehoe, CTTI

10:35 a.m. Session III: Ensuring a DHT-derived Endpoint is Pivotal Trial Ready

Session Facilitator/Moderator: Tom Switzer, Genentech
Session Objectives:

- Discuss the process and data needed to support that a DHT-derived endpoint is ready for pivotal clinical trials
- Explore how to link a DHT measure to a relevant, clinically important outcome
- Describe how to demonstrate meaningful within patient change using a DHT

10:40 a.m. Use of Digital Health Technologies in Patient-Focused Drug Development: A Regulatory Perspective
Lili Garrard, FDA/CDER

Considerations for Deriving a DHT-Based Endpoint Measure for use in Pivotal Trials
Sonya Eremenco, Critical Path Institute

Digital Measures in Clinics-What Matters?
Jörg Goldhahn, ETH Zurich

11:10 a.m. Break Out Groups

- Discuss how to demonstrate endpoint readiness for regulatory decision making
- Discuss how to demonstrate the meaningfulness of change in the endpoint
- Potential questions include:
  - How do we appreciate the level of change needed to make a difference in a patient's daily life?

- What data is needed to show differences in measuring change?
  - within a population (different degrees of disease)
  - within an individual (individual treatment effect)
- What data is needed when the clinical measurement comparator (i.e. anchor/reference measure) is suboptimal?

11:45 a.m. **Break**

11:50 a.m. Summary of Break Out Groups
Break Out Group Facilitators

12:15 p.m. **Session IV: Advancing Adoption through Collaboration**

Session Facilitator/Moderator: Alicia Staley, Medidata
Session Objectives:

- Explore the incentives, disincentives, and opportunities to collaborate on DHT-derived endpoint development
- Address what existing evidence can be leveraged and at which phases of the endpoint development process
- Explore ways to expand digitally derived endpoints used in one context to another context

12:20 p.m. Leveraging Evidence Presentation
Kai Langel, Janssen

12:35 p.m. Open Source for Digital Endpoints: Open Wearables Initiative
Geoff Gill, Shimmer Americas

12:50 p.m. Q&A

1:00 p.m. Polling for How We Can Collaborate and What We Can Leverage
All attendees
Potential questions include:
- What are the main incentives to collaboration when developing novel DHT-derived endpoints? What are the main disincentives?
- Who should be collaborating to advance the adoption of novel DHT-derived endpoints?
- What are the top three elements where reuse could most benefit the endpoint development process?

1:25 p.m. **Closing Comments and Unified Call to Action**
Lindsay Kehoe, CTTI

1:30 p.m. **Adjourn**