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.
<table>
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<th>Time</th>
<th>Session</th>
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<td>10:30 a.m.</td>
<td><strong>Welcome &amp; Opening Remarks</strong></td>
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| 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

10:35 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

For more information, contact the Novel Endpoints Acceptance Project Manager, Lindsay Kehoe at Lindsay.kehoe@duke.edu, or visit http://www.ctti-clinicaltrials.org.