

EXPERT MEETING SUMMARY | JULY 27-28, 2021



BACKGROUND

As part of its 2017 Novel Endpoint work, CTTI created recommendations for the selection and development of endpoints that use digital health technologies (DHTs) for data capture. Despite the increased use of DHTs in trials since then, few examples of a digitally-derived primary endpoint exist today.

CTTI is now working to advance the adoption of meaningful, DHT-derived novel endpoints. This meeting explored the challenges, opportunities, and solutions for using DHT-derived endpoints, with the goal of creating recommendations and resources to increase their use as key endpoints in clinical trials for labeling claims.



MEETING OBJECTIVES

During the meeting with relevant experts and key stakeholders—including investigators, patients, regulators, technology experts, sponsor representatives, and other groups—CTTI and attendees:

- Identified the barriers and discussed solutions to the adoption of DHT-derived endpoints into pivotal trials
- Discussed the data needed to demonstrate that a DHT-derived endpoint is ready for a pivotal trial
- Explored how collaboration and other new efforts can advance the adoption of DHT-derived endpoints



MEETING THEMES

- **The Time is Now.** Digitally derived endpoints have the ability to capture information that is more reflective of how patients feel and function in their day-to-day lives—but many factors hinder their acceptance. Collaborative partnerships and solutions that increase the understanding and use of DHT-derived endpoints are needed to make this happen.
- **Fit-for-Purpose is a Must.** DHT-derived endpoints need to be based on the specific context of use and validly measure a concept of interest in a way that is accurate, interpretable, and not misleading.
- **“Meaningful” in More than One Way.** To develop a DHT-derived endpoint, have the community—including patients and clinicians—discuss what constitutes “meaningful” change. A meaningful measure should show treatment benefit as well as clinical benefit.
- **Engage Early and Often.** Engage patients, investigative site personnel, and the FDA early and often when planning; involve biostatisticians and data scientists, as appropriate, in decisions regarding protocol design, data collection, analysis, and interpretation.
- **Validate, Validate, Validate.** It’s important to remember that validating a device is a separate, yet parallel, process from validating the clinical measure. It is also especially important to demonstrate analytical validation of DHT algorithms within the specific patient populations.



NEXT STEPS

Meeting participants identified distinct and actionable suggestions that the CTTI project team can use as the foundation for its next step—developing recommendations and resources to drive the practical use of novel, digitally derived endpoints in clinical trials.



ADDITIONAL RESOURCES

- **View** the meeting materials, including agenda, participant list, and presentations.
- See a sneak peek of CTTI's Novel Endpoint Acceptance recommendations and resources during a **[webinar on Oct. 5.](#)**
- For more information, please contact Lindsay Kehoe at **Lindsay.Kehoe@duke.edu**.



ABOUT THE CLINICAL TRIALS TRANSFORMATION INITIATIVE (CTTI)

The Clinical Trials Transformation Initiative (**[CTTI](#)**), a public-private partnership co-founded by Duke University and the FDA, seeks to develop and drive adoption of practices that will increase the quality and efficiency of clinical trials. Bringing together organizations and individuals from across the enterprise CTTI is transforming the clinical trials landscape by developing evidence-based solutions to clinical research challenges.



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