REAL WORLD DATA AND EVIDENCE IN THE EVALUATION OF MEDICAL PRODUCTS

Expert Meeting

CLINICAL TRIALS TRANSFORMATION INITIATIVE

EXPERT MEETING DETAILS | JUNE 12-13, 2018

BACKGROUND

The use of real world evidence (RWE) in randomized studies holds great promise for improving the quality and efficiency of clinical trials. RWE—the analysis of real world data (RWD) from sources such as electronic health records (EHR) and insurance claims databases—may provide a more complete picture of patient experiences, reduce costs and burdens, and offer insights into important questions that would otherwise be impossible to answer.

Despite growing interest in using RWD, there is still a need to develop effective methods and approaches to leverage and evaluate the utility of EHR and claims data, as well as other RWD sources to enhance clinical research. In addition, lack of awareness and consensus among stakeholders about valid approaches to leverage these data sources has slowed adoption. To address this need, CTTI is conducting in-depth research to develop actionable recommendations that clarify the best approaches to utilize RWD, specifically EHR and claims data, in clinical trials.

MEETING OBJECTIVES

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

- Presented findings from evidence gathering activities;
- Discussed direction for the appropriate use of EHR and payment claims to support planning and execution of RCTs;
- Identified barriers and potential solutions to generating RWE for regulatory submissions from these RWD sources;
- Described what recommendations and resources CTTI should develop to equip change agents to increase appropriate use of RWD in RCTs, including to support regulatory submissions.

MEETING THEMES

- **Using RWE Could be a Major Step Forward...But We Need to Recognize its Limitations:** The use of RWD and RWE in RCTs has the potential to increase trial efficiency, reduce cost, decrease patient burden, improve generalizability by ensuring a more representative population, and enhance rigor by enabling the collection of long-term data. At the same time, it is important to be aware of potential challenges, such as inconsistent data quality, reliability, and depth; the need for robust validation approaches; and the difficulty of collecting subtle outcomes. Given all of this, it is important to carefully review data for accuracy and establish a clear algorithm capable processing the data and identifying any potential issues.

- **We Need a Way Forward:** The enterprise is eager to use RWD and RWE in clinical trials, but the absence of recommendations, guidance, and "how to" materials is stalling adoption.

- **Data Source Interoperability is Necessary:** Different RWD sources exhibit different strengths and weaknesses. While administrative claims data is robust in terms of being able to capture data longitudinally, EHR data has more richness and depth. As a result, linking multiple data sources is critical for leveraging all strengths and improving both the depth and breadth of available data.

- **Patient Engagement is Still Essential:** Stakeholders must remember that the use of RWD and RWE does not replace the need for direct interaction with participants to ensure data is timely and accurate. Participant consent should be carefully considered in trials using RWD and RWE—so that participants are made aware of how their data will be used and managed—and fostering a culture of data sharing is important.

- **The FDA Continues to Explore the Utility of RWD and RWE:** According to many FDA attendees, the agency supports the view that the use of RWD can help inform study design and identify patient populations among other enhancements; however, expanding the use of EHR and claims data to support regulatory decision-making requires further evaluation. More specifics are needed to determine where routine data collection can be used to measure certain outcomes and where more validation is needed. FDA is currently piloting the use of RWD/RWE in multiple collaborative projects.

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 for incorporating RWD and RWE into randomized trials for regulatory submission. Resources under development include:

- "How to" guides and recommendations on using EHRs and claims data to:
  - Analyze the impact of varying eligibility requirements;
  - Determine site selection; and
  - Recruit and enroll patients.
- Template consent language for RWD-sourced research.

ADDITIONAL RESOURCES

- **Meeting materials**, including agenda, participant list, and presentations
- Read more about CTTI's **Real World Evidence Program**
- For more information, please contact Gerrit Hamre at **gerrit.hamre@duke.edu**

ABOUT CTTI

The Clinical Trials Transformation Initiative (CTTI), a public-private partnership co-founded by Duke University and the U.S. Food and Drug Administration, seeks to develop and drive adoption of practices that will increase the quality and efficiency of clinical trials. Comprised of more than 80 member organizations, CTTI is transforming the clinical trials landscape by developing evidence-based solutions to clinical research challenges. Learn more about CTTI at **www.ctti-clinicaltrials.org.**

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