



Real World Data and Evidence in the Evaluation of Medical Products

Agenda of the Multi-Stakeholder Expert Meeting held June 12-13, 2018
Double Tree by Hilton Hotel | 8120 Wisconsin Ave, Bethesda, MD 20814

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

MEETING SCOPE:

- ▶ Real World Data (RWD) are data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources.
- ▶ Real World Evidence (RWE) is the clinical evidence regarding the usage, and potential benefits and risks, of a medical project derived from analysis of RWD.¹
- ▶ The focus of this meeting and project is to explore the appropriate use of electronic health records and payment claims (RWD) in randomized clinical trials (RCTs) to generate RWE in support of regulatory decision-making.²

MEETING OBJECTIVES:

- ▶ Present findings from evidence gathering activities.
- ▶ Identify barriers and potential solutions to generating RWE for regulatory submissions from these RWD sources.
- ▶ Describe what recommendations and resources CTTI should develop to equip change agents to increase appropriate use of RWD in RCTs, including to support regulatory submissions.

¹ Guidance for Industry and Food and Drug Administration Staff, *Use of Real-World Evidence to Support Regulatory Decision Making for Medical Devices*, August 2017

² CTTI RWE project team members recognize the interest and potential of retrospective observational studies, including retrospective database analysis of claims data and electronic health records, as well as registry-based trials (which were the topic of a recent CTTI project and activities). However, this meeting will focus on RCTs that leverage RWD for study planning, recruitment, enrollment or collection of study outcomes. Also, note the focus of this meeting is discussion of data collected from mobile clinical trials (MCT) and wearables, which is the focus of CTTI's MCT Program.

JUNE 12, 2018

8:00 a.m. **Breakfast** (*Provided in the Ballroom, Level 2*)

8:30 a.m. **Welcoming Remarks**

Introduction to the Clinical Trials Transformation Initiative (CTTI)
Pamela Tenaerts, CTTI

8:45 a.m. **Session I: Project Scope, Overview, and Interview Findings**

Session Facilitator: Gerrit Hamre, CTTI

Session Objectives:

- ▶ Describe the scope of CTTI's RWE project in the context of the broader field of RWE.
- ▶ Frame meeting objectives in terms of developing practical models and operational guidance for use of RWD to enhance RCTs.
- ▶ Present and discuss findings from the RWE project evidence-gathering activities.

8:45 a.m. Issue, Project Overview, and Meeting Objectives
Gerrit Hamre

9:00 a.m. Framework Focus: Use of RWD Sources to Facilitate RCTs
David Thompson, Syneos Health

9:15 a.m. Qualitative Interview Findings
Jack Sheehan, Johnson & Johnson/Janssen

9:30 a.m. Open Group Discussion

10:00 a.m. **Break**

10:15 a.m. **Session II: Use of RWD in Pre Study Planning and Study Set up**

Session Facilitator: Jane Perlmutter, Patient Representative

Session Objectives:

- ▶ Discuss examples of RWD use in pre-study planning and study set up of RCTs.
 - Defining and refining cohort
 - Assessing usability of inclusion and exclusion criteria
 - Generating hypotheses
 - Modeling outcomes (i.e. event rates)
- ▶ Modeling enrollment forecasts. Discuss opportunities and challenges for increased RWD use in pre-study planning and study set up.

Presenters

10:25 a.m. *Kevin Haynes, HealthCore*

10:45 a.m. *Ben Gutierrez, GlaxoSmithKline*

11:05 a.m. *Aliza Fink, Cystic Fibrosis Foundation*

11:25 a.m. Open Group Discussion

12:15 p.m. **Lunch** (*Provided*)

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1:00 p.m. Session III: Use of RWD in Study Recruitment and Enrollment

Session Facilitator: Scott Evans, Society for Clinical Trials

Session Objectives:

- ▶ Discuss examples of RWD use in study recruitment and enrollment.
 - Incorporating screening criteria for contacting, scheduling, or enrolling patients
 - Embedding clinician prompts regarding assessing patient eligibility
 - Site/investigator selection
- ▶ Discuss opportunities and challenges to increased RWD use in study recruitment and enrollment.

Presenters

- 1:10 p.m. *James Hamrick, Flatiron Health*
1:25 p.m. *David Thompson, Syneos Health*
1:40 p.m. Open Group Discussion

2:00 p.m. Break

2:15p.m. Session IV: Use of RWD in Study Conduct

Session Facilitator: Lesley Curtis, Duke University

Session Objectives:

- ▶ Discuss examples use of RWD use in study conduct.
 - Capturing study specific efficacy and safety data
 - Leveraging data on alerts, checks for hospitalizations, AEs, and event rates
 - Monitoring changes in patient treatment
 - Validating endpoints (i.e. cutoffs)
 - Evaluating historical or concomitant controls
- ▶ Discuss opportunities and challenges to increased RWD use in study conduct.

Presenters

- 2:25 p.m. *Michael Lu, Genentech, A member of the Roche Group*
2:45 p.m. *Sarah Leatherman, U.S. Department of Veterans Affairs*
3:05 p.m. *Brad Hammill, Duke University*
3:25 p.m. Open Group Discussion

4:15 p.m. Session V: Dialogue with Regulators on the Use of RWD in RCTs

Session Moderator: Pamela Tenaerts, CTTI

Session Objectives:

- ▶ Discuss regulatory perspectives on using RWD sources in RCTs.
- ▶ Discuss relevant components of 21st Century Cures Act and PDUFA VI Commitment Letter.

Panelists

Mark Levenson, FDA CDER
Peter Marks, FDA CBER
Robert Temple, FDA CDER
Karen Ulisney, FDA CDRH

5:15 p.m. Adjourn to Dinner Reception (RSVP Requested)

JUNE 13, 2018

8:00 a.m. **Breakfast** (*Provided*)

8:30 a.m. **Session VI: Day One Recap and Discussion of Priority Insights**

Session Moderator: Jacqueline Corrigan-Curay, FDA CDER

Session Objectives:

- ▶ Briefly revisit framing and scope of project work.
- ▶ Discuss and prioritize themes from previous day.
- ▶ Identify areas in need of greater discussion.

Panelists

Lesley Curtis, Duke University

Scott Evans, Society for Clinical Trials

Jane Perlmutter, Patient Representative

9:00 a.m. **Session VII: Breakout Groups Actionable Opportunities for Transformative Change**

Breakout Instructions

Gerrit Hamre, CTTI

Group 1: Use of RWD in RCTs to Generate RWE in Pre-Study Planning & Set Up

Group 2: Use of RWD in RCTs to Generate RWE in Study Recruitment & Enrollment

Group 3: Use of RWD in RCTs to Generate RWE in Study Conduct

10:45 a.m. **Break**

11:00 a.m. **Session VIII: Addressing Use of RWE for Regulatory Decision Making**

Session Moderator: Gerrit Hamre

Session Objectives:

- ▶ Breakout teams present and engage all attendees in discussion on consensus approaches / recommendations / best practices / guiding principles for their assigned topics.
- ▶ Discuss how attendees would like to see this information reported in CTTI recommendations and resources.

12:00 p.m. **Closing Comments**

12:00 p.m. **Highlights, Next Steps, and Adjourn**

Gerrit Hamre

Lunch (*Provided*)