THE FORUM
For Collaborative Research℠

Translating Real-World Data into Real-World Evidence Project

Webinar 2: Statistical Analysis and Data Quality

Monday, November 8, 2021

Berkeley Public Health

Agenda

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Translating Real-World Data into Real-World Evidence Project
Webinar 2: Statistical Analysis and Data Quality

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November 8, 2021
9:00am-11:00am ET

Meeting Open		
	Moderator: *Veronica Miller, Forum for Collaborative Research*	
9:00am ET	Welcoming Remarks	*Veronica Miller, Forum for Collaborative Research*
9:05am ET	Case Study: Prograf Approval for Lung Transplants	*Ryan Jung, FDA*
	Challenges in Statistical Approaches using Real-World Data	*Nigel Hughes, Janssen Pharmaceuticals/EHDEN*
	Innovative Statistical Approaches	*Mark van der Laan, Center for Targeted Learning* *Rima Izem, Novartis*
9:45am ET	Challenges of Maintaining Data Collection and Continuity Across Subpopulations and Age groups a. From children into adulthood b. From metabolic disorders into transplant centers	*Donna Cruz Jones, Patient Advocate* *Pam Vig, Mirum Pharma* *Ray Huml, Syneos* *Rima Izem, Novartis*
10:25am ET	Panel Discussion & Audience Q&A	*All*
10:50am ET	Conclusions/ Next Steps	*Veronica Miller, Forum for Collaborative Research*
Adjourn Webinar		

Translating Real-World Data into Real-World Evidence Project Webinar

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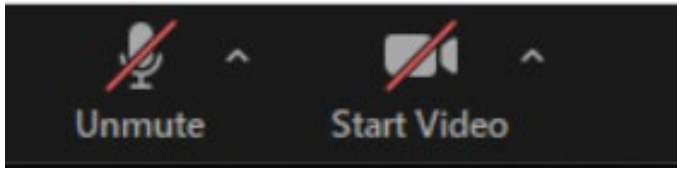
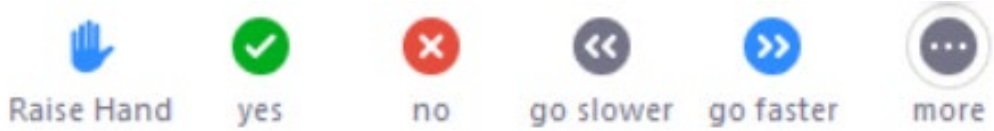


- Audience members will be muted on entry
- Clarifying questions may be answered by the presenter
- Discussion will take place after the presentations
- To ask questions or make a comment:
  - If generally applicable to the session, please use the Q&A or chat function on Zoom and send to everyone
  - Or use the raise your hand function



Rules of the [Zoom] Room

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- **Presentations, discussions, comments, and questions are not for attribution**
  - Participants speak as individuals and express views that may not represent those of their organizations
  - Recording by participants is not permitted. Slides will be available on our website pending speaker permission.
- **Participation Reminder (Forum Operating Principles)**
  - We restrict participation to experts with the necessary scientific knowledge from organizations or entities with a clear commitment to advancing the diagnostic and therapeutic field of the disease area of the project they participate in, whether they be from government, academic, industry or community. Only in this way can we achieve the effectiveness and productivity needed to add value to the field. The Forum is not a venue for marketing and/or investment experts.

Webinar 1: US and European Stakeholder Perspectives Summary

- Webinar 1 featured patient, industry, academic, and regulatory experts from the U.S. and Europe.
- The webinar began with an overview of the FDA's Real-World Evidence Program, FDA's approach to evaluating real-world evidence, definitions of real-world data and real-world evidence, and examples of real-world data and evidence including the ICAREdata project and the approval of Prograf.

Webinar 1: US and European Stakeholder Perspectives Summary

- The second presentation of Webinar 1 provided a summary of the European Medicines Agency approach to real-world evidence including:
  - the establishment of the Big Data Steering Group, which was formed by the European Medicines Agency and Head of Medicines Agency. Its workplan has 10 recommendations
  - the use of real-world evidence in marketing authorization application and extension of indications, and
  - the use of studies in-house such as EMA studies on electronic health databases, commission studies procured through the EMA framework, and the use of DARWIN EU (starting in 2022).

Webinar 1: US and European Stakeholder Perspectives Summary

- The third presentation discussed lessons learned from the use of real-world data as external controls, covering topics including the process of harmonization, the assessment of feasibility, highlighted the GLOBAL Primary Biliary Cholangitis Study Group (2012) and GALA: The Global Alagille Alliance Study (2018) as successful uses of real-world data and the importance of creating easier pathways for collaboration to improve patient care.
- The final presentation of Webinar 1 focused its discussion on fit-for-purpose data. Key takeaways included (1) registration of a new drug requires a well-controlled trial and (2) the Estimands Framework (ICH E9) is useful to consider especially in the context of rare diseases with small populations.
- The panel discussion demonstrated how collaboration between academicians, patients or patient advocates, industry, and regulators will continue to inform the way we think about clinical trial designs and real-world evidence in its collection to be used as fit-for-purpose data.

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