THE FORUM
For Collaborative Research℠

Translating Real-World Data into Real-World Evidence Project

Webinar 1: US and European Stakeholder Perspectives

Tuesday, July 20, 2021

Berkeley Public Health

THE FORUM
For Collaborative Research℠

Introduction & Forum Updates

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Forum for Collaborative Research

Berkeley Public Health

Agenda

Meeting Open		
	Moderators: *Veronica Miller, Forum for Collaborative Research & Sandra Lehrman, Lehrman Family Research Fund*	
10:00am EST	Welcoming Remarks & Program Introductions	*Veronica Miller, Forum for Collaborative Research*
10:10am EST	Discussion with Leadership Representatives from US and Europe a. Overarching Principles for Fit-for-Purpose Data b. Data Quality and Effective Standards for Regulatory Process c. Practical Application of Real-World Evidence	*John Concato, FDA* *Ruby Mehta, FDA* *Gianmario Candore, EMA/COMP*
10:40am EST	Lessons Learned from Use of Real-World Data as External Controls	*Bettina Hansen, University of Toronto*
10:55am EST	Fit-for-Purpose Data	*Jerry Vockley, University of Pittsburgh* *Satrajit Roychoudhury, Pfizer*
11:15am EST	Multi-Stakeholder Panel a. The Role of Data at Different Stages of Drug Development: From Proof of Concept to Confirmatory b. Translation of Real-World Data from Various Sources to Fit-for-Purpose Evidence	*Jerry Vockley, University of Pittsburgh* *Satrajit Roychoudhury, Pfizer* *John Concato, FDA* *Ruby Mehta, FDA* *Gianmario Candore, EMA/COMP* *Violeta Stoyanova, EMA/COMP* *Bettina Hansen, University of Toronto* *Ruth-Anne Pai, PSC Partners Seeking a Cure*
11:45am EST	Preview Webinar 2: Statistical Analysis and Data Quality	*Rima Izem, Novartis*
11:50am EST	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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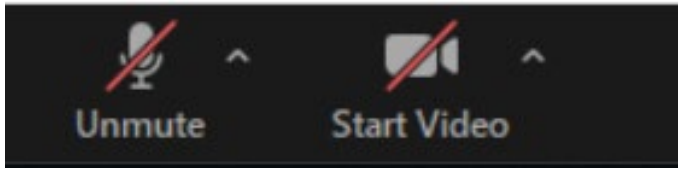
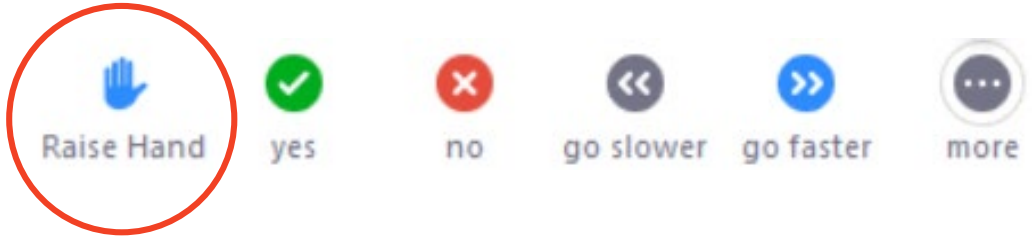
- Audience members will be muted on entry



- 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



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Rules of the [Zoom] Room

- **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, academia, 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.

Translating Real-World Data into Real-World Evidence Project

- This project will explore overarching prerequisites for fit-for-purpose data, main challenges and implications, and the statistical significance of related innovations. Expected outcomes include the formation of new working groups to carry on the work of the overarching project by addressing priority areas identified.

- Webinar 1: US and European Stakeholder Perspectives is the first in a series of cross Forum webinars in 2021. It will focus on regulatory perspectives, lessons learned, fit-for-purpose data and a panel discussion.

- Webinar 2: Statistical Analysis and Data Quality will be scheduled for August or September.

Overview of the Liver Forum, PSC Forum and Rare Diseases Forum

- **What:** Forums provide a platform for ongoing multi-stakeholder dialogue to identify barriers, prioritize research and identify solutions to accelerate therapeutic development
- **How:** provide a neutral, independent, safe space for discussion and deliberation across stakeholder groups
  - Focus on developing consensus, increasing synergy and collaboration, and reducing duplication and uncertainty
  - Ongoing working group activity throughout the year anchored by larger project events
  - Active & engaged participation

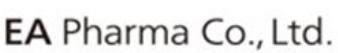
Liver Forum

- **Aim:** to advance the regulatory sciences for the treatment of NAFLD/NASH and liver fibrosis by providing an independent and neutral venue for ongoing multi-stakeholder dialogue.
- LF projects of interest to this webinar series:
  - Pediatric liver diseases
    - Small populations, lack of biomarkers/endpoints, diverse natural history studies
  - NASH placebo-arm database
    - Not a rare disease – hope that this unique version of a natural history study can contribute to better understanding/ cleared definition of endpoints

PSC Forum

- Rare/ heterogenous disease
- Focus on defining appropriate endpoints
- Working Groups
  - PSC biomarkers
  - Role of patient-reported outcomes in PSC drug development
  - Cross-talk w LF on pediatric cholestatic disease
    - https://forumresearch.org/liver-forum/pediatric-cholestatic-disease-program/1709-pediatric-cholestatic-disease-program
- Collaboration Paediatric Cholestasis Workshop and in-person meeting in Spring 2022.

Liver Forum & PSC Forum Sponsors



Liver Forum & PSC Forum Sponsors

Rare Diseases Forum

- Focus on “rare diseases”
  - Inborn errors of metabolism, neurodegenerative diseases, rare/ultrarare
- Recent publication: *The Use of External Controls in FDA Regulatory Decision Making*
  - Jahanshahi, M., Gregg, K., Davis, G., Ndu, A., Miller, V., & Vockley, J. et al. (2021). The Use of External Controls in FDA Regulatory Decision Making. *Therapeutic Innovation & Regulatory Science*. doi: 10.1007/s43441-021-00302-y
- Manuscripts in preparation
  - Lessons Learned from Clinical Development of Recent Therapies for Rare Disorders
  - Scientific and Regulatory Considerations for Complex Innovative Trial Designs for Gene Therapy and Rare Disease Drug Development
  - Exploring the Potential Contribution of Innovative Tools, Technologies, and Data Analytics to Assess Clinical Outcomes
  - Borrowing of External Controls and Usage of Bayesian Methods in Rare Diseases Studies

Rare Diseases Forum Sponsors

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Webinar 1: US and European Stakeholder Perspectives

- Organized to elucidate stakeholder perspectives including regulators, industry, academics, and patient advocates
- Explore shared knowledge gaps across disease areas with experts from the Liver Forum, PSC Forum and Rare Disease Forum
- Identify the application and challenges of real-world data and real-world evidence to promote best practices in clinical trial design
- Examine data quality and standardization throughout stages of drug development and the use of external controls to determine efficacy in investigational treatments

Translating Real-World Data into Real-World Evidence Project: Some Research Questions

- How can registries be used to bridge the gap between academia and industry to promote cross sector efforts?
- What endpoints could be used across diseases and how do we measure them?
- How can the continuity between pediatric and adult research be improved?
- What are the optimal statistical approaches?
- When values should be used to promote data quality?
- How can bias be reduced by using analytic metrics?