Navigating Challenges and Seizing Opportunities: Leveraging Multiple RWD Sources in External Control Arms for HTA and Regulatory Decision-Making

November 13, 2023

Today's Presenters

CHRIS CAMERON

Chief Scientific Officer at EVERSANA & Moderator

NICOLLE GATTO

Chief Science Officer, AETION – RWE research organization perspective

QIUFEI MA

Senior Director, Health Economics and Outcomes Research, Regeneron Pharmaceuticals Inc & industry sponsor perspective

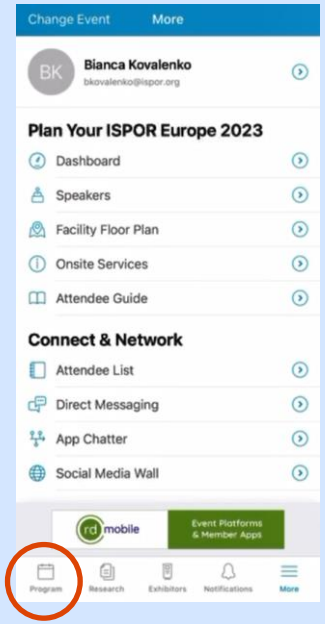
UWE SIEBERT

Professor and Head of Department at UMIT TIROL & academia/HTA reviewer perspective

How to Participate in Polling Questions

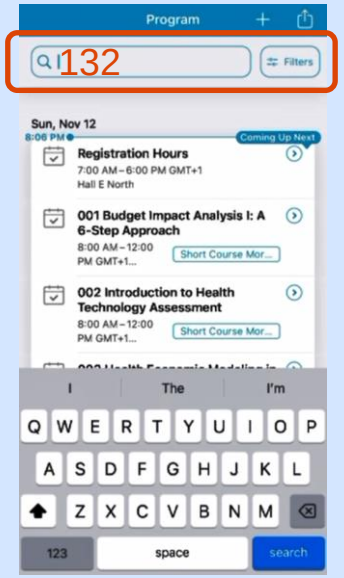
- Download the ISPOR mobile app
- Navigate to **Session 132** to participate!
- Polling questions will be used for knowledge checks throughout the sessions

1



Tap on “Program”

2



Type “132” in the search bar

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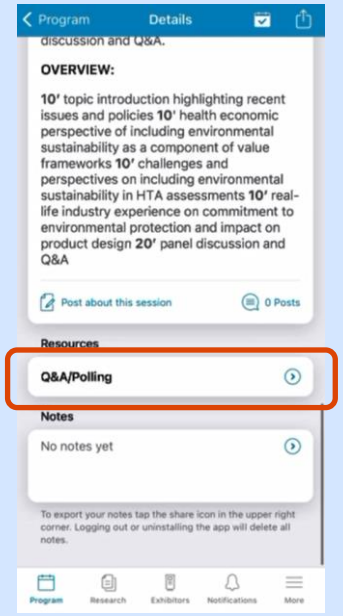


At the bottom of the session page, tap “Q&A/Polling”

4

Select the polling question to submit your answer

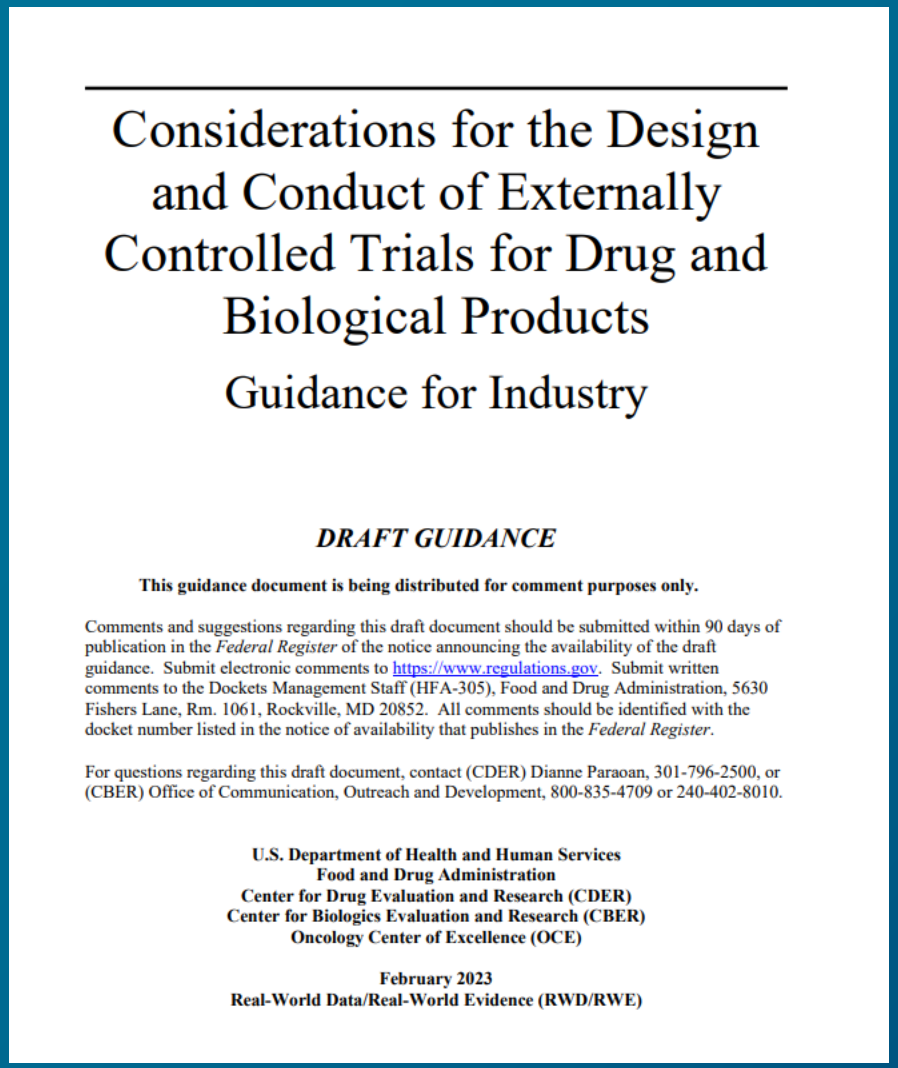
Discussion Group

Navigating Challenges and Seizing Opportunities: Leveraging Multiple RWD Sources in External Control Arms for HTA and Regulatory Decision-Making

Monday, 13 November 2023
(15:15 – 16:15)

We will be hosting a subsequent Discussion Group as part of the Spotlight Series.

Please join us in the Discussion Lounge in Hall E North in Discussion Group B



Considerations for the Design and Conduct of Externally Controlled Trials for Drug and Biological Products

Guidance for Industry

DRAFT GUIDANCE

This guidance document is being distributed for comment purposes only.

Comments and suggestions regarding this draft document should be submitted within 90 days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit electronic comments to https://www.regulations.gov. Submit written comments to the Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions regarding this draft document, contact (CDER) Dianne Paraoan, 301-796-2500, or (CBER) Office of Communication, Outreach and Development, 800-835-4709 or 240-402-8010.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)
Oncology Center of Excellence (OCE)

February 2023
Real-World Data/Real-World Evidence (RWD/RWE)

“…for decades FDA has recognized the potential value of other types of controls, including historical controls as a type of external control. Clinical trials using these other types of controls can, when appropriate, serve as the adequate and well-controlled clinical investigations generally required to provide substantial evidence of effectiveness under section 505(d) of the Federal Food, Drug, and Cosmetic Act (FD&C Act).

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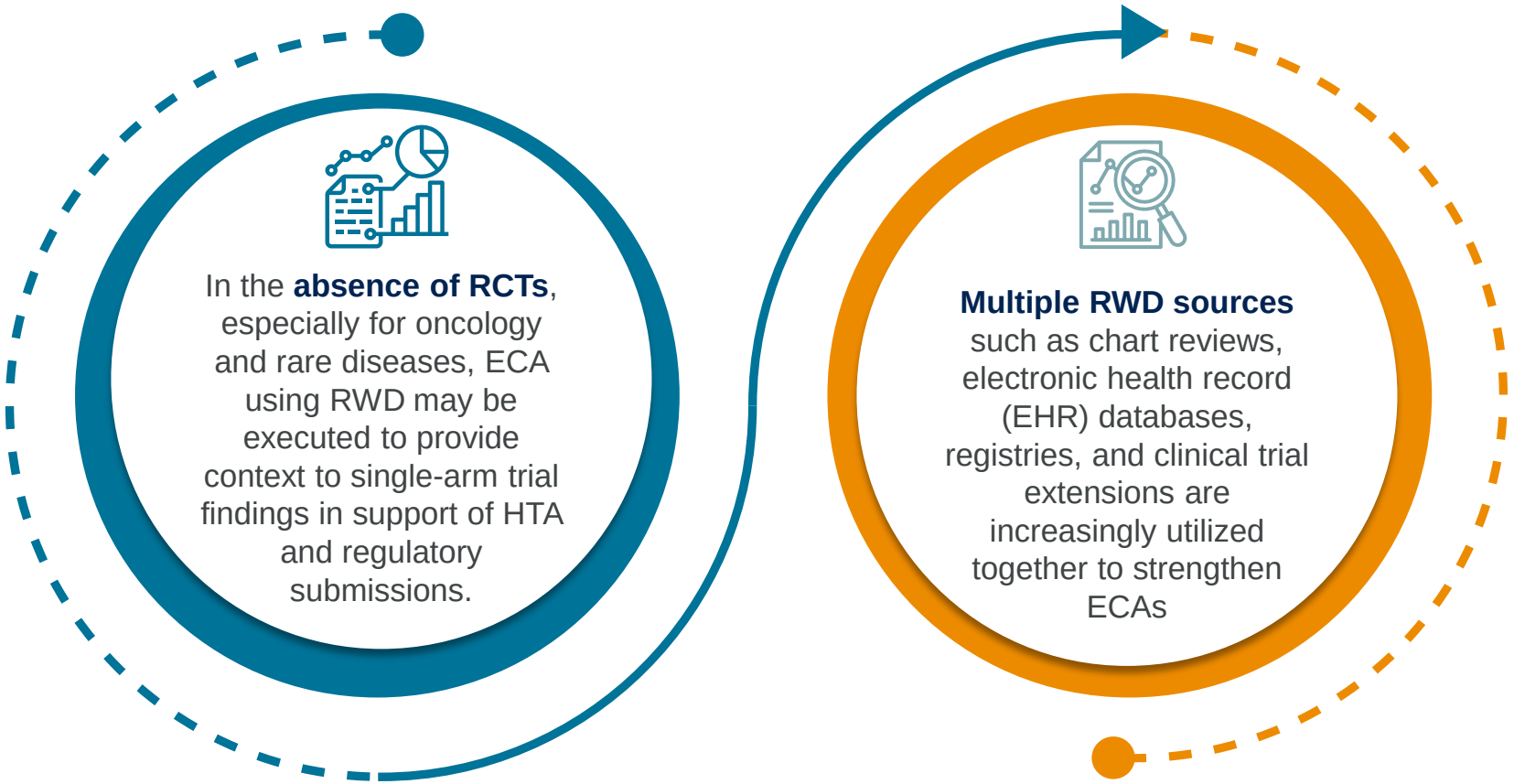

Opportunity:

Multiple RWD sources offers valuable insights into treatment effects across diverse populations, geographies, data types, and timeframes. However, challenges persist in aligning trial criteria, treatment modalities, endpoints, and confounders across different data sources.

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International experts will contribute their perspectives on how leveraging and harmonizing multiple RWD sources can enhance the evidentiary requirements of global HTA and regulatory bodies

What challenges and opportunities arise when harmonizing multiple RWD sources for creating ECAs in HTAs and regulatory submissions?

How can we address emerging RWE guidance and enhance acceptance of ECAs by harmonizing multiple RWD sources?

Polling Question 1

Do you have experience with External Control Arm (ECA) submissions to Health Technology Assessment (HTA) and/or regulatory bodies?

A. Yes – Regulatory only

B. Yes – HTA only

C. Yes – Both HTA and Regulatory

D. No

Abbreviation: ECA = External Control Arm; HTA = Health Technology Assessment

Polling Question 2

For those that have submitted an ECA to regulatory or HTA, did that ECA meet its intended use?

A. Yes

B. No

Abbreviation: ECA = External Control Arm; HTA = Health Technology Assessment

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PANEL PRESENTATIONS

Polling Question 3

In your opinion, can leveraging multiple RWE sources enhance acceptance of ECAs by regulatory and HTA bodies?

A. Yes

B. No

Abbreviation: ECA = External Control Arm; HTA = Health Technology Assessment; RWE = Real-World Evidence

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PANEL DISCUSSION

QUESTIONS?

Polling Question 4

Can regulatory and HTA bodies do more to enhance the use of ECAs leveraging multiple RWE sources?

A. Yes

B. No

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