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

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Today’s Presenters

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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
3. 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
...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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In the **absence of RCTs**, especially for oncology and rare diseases, ECA using RWD may be executed to provide context to single-arm trial findings in support of HTA and regulatory submissions.

Multiple RWD sources such as chart reviews, electronic health record (EHR) databases, registries, and clinical trial extensions are increasingly utilized together to strengthen ECAs.

**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?
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
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
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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