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Use of RWD in Pre-Study Planning and Study Set Up

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Disclaimer

The views and opinions expressed in this presentation are those of the individual presenter and do not necessarily reflect the views of the Clinical Trials Transformation Initiative.

Session Objectives

- Provide example uses of RWD sources to generate RWE in pre-study planning and study set up of RCTs.
- Discuss the opportunities and challenges for increased RWD use in pre-study planning and study set up.

Session Presenters

 Kevin Haynes, HealthCore

 Ben Gutierrez, GlaxoSmithKline

 Aliza Fink, Cystic Fibrosis Foundation

Use of RWE in Planning RCTs

- ▶ Select appropriate populations
 - Sub-populations
 - Patients at risk
- ▶ Determine endpoints
- ▶ Understand burden of disease
- ▶ Determine appropriate comparator
 - Establish Standard of Care (SoC)
- ▶ Calculate sample size based on comparator baseline
- ▶ Identify potential study sites
- ▶ Estimate accrual time-line
- ▶ Assess overall feasibility

RWE Opportunities

- ▶ Improve the scientific rigor, scope, and healthcare benefits of clinical research by:
 - Assessing the comparability of evidence from clinical trials and RWD to determine generalizability/potential bias in RWE.
 - Collecting more generalizable data and conducting more agile, faster, and cheaper studies.
 - Leveraging the size and scope of RWD sources to improve researchers' ability to impute missing data and address biases.

RWE Gaps & Needs

- Tools for working with live data in trial planning.
- Standards for linking patient data across healthcare systems and data sources.
- Modeling and simulation tools to better understand what is and is not feasible for clinical trials to assess.

RWE Considerations

“I think the biggest area where you have to be careful in is what questions you ask with respect to how do you determine the endpoint? And if you determine an endpoint that needs input from the patient, for example, that’s problematic because you have to reach the patient.”



“Much of our core business actually is designing and conducting post-marketing safety studies using preempted and, more recently, EHR data. Many of these studies are in support of... post-marketing regulatory reporting requirements. So, that includes requirements for the FDA as well as for European agencies.”



Discussion Questions

- In addition to examples discussed by the speakers, where else have you found RWD helping in informing pre-study planning and study set up?
- What have been your key challenges? How might you overcome these challenges?
- To what extent do you expect to use RWD to inform pre-study planning and study set up in the future?
- What data sources do you intend to use?

THANK YOU.



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