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Improving healthcare decisions

DO WE STILL NEED RANDOMIZED CONTROLLED TRIALS?

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Presenters

- Moderator – Lucinda Orsini, Associate Chief Science Officer, ISPOR
- C. Daniel Mullins - University of Maryland Baltimore, School of Pharmacy
- Leanne Larson - Vice President/Global Head, RWE, Parexel International
- Jeroen P Jansen - Chief Scientist, Precision HEOR

Why we love Randomized Controlled Trials

- Random Treatment Assignment
- Ability to blind the patient and trialist to treatment group
- Interventions are held constant except for the experimental treatment
- Endpoints
  - well understood
  - the study is designed to measure that one specific outcome
- Why? To eliminate bias as much as possible

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Challenges to Using RWE for decision-making

- Considerations for making causal inference from studies that are not randomized
  - Underlying data quality
  - Data dredging
  - Lack of transparency of research questions/objectives, data set choice, and a priori analysis plans
  - Lack of visibility into the universe of studies conducted versus published studies, particularly for comparative effectiveness

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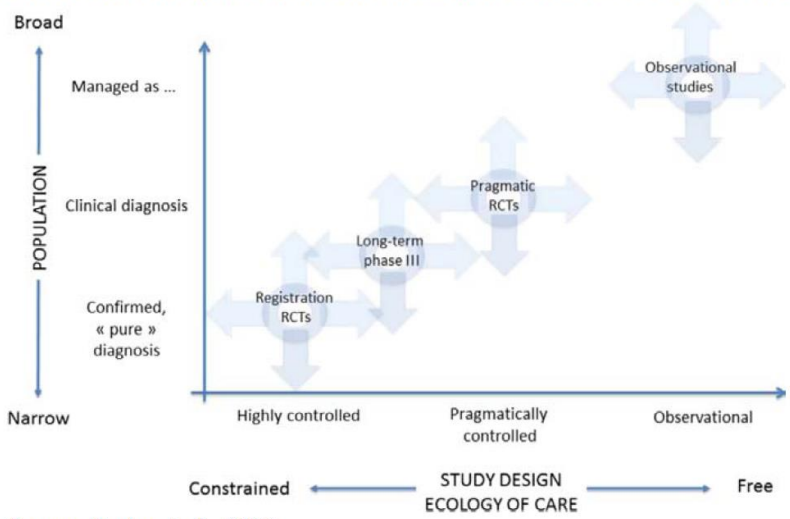
Figure 1: Conceptual framework of therapeutic research by study design



Source: Roche et al., 2013

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How RCTs compare to RWE

Clinical Trials Answer Benefit/Risk

- Efficacy – does it work:
  - Narrowly defined population
  - Under very controlled conditions
  - Versus: placebo or one active comparator
  - For the duration of the trial
- Safety
  - Can {known} Side Effects and Adverse Events be managed under controlled conditions

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Real-World Evidence Answers:

- Effectiveness – does it work in practice?
  - In broader populations
  - Under uncontrolled conditions
  - Over the long term
  - Versus all current standards of care
- Safety – is it safe in practice?
  - Are {known} side effects
    - reported by patients
    - Treatable in clinical practice
  - Unknown side effects
- Adherence/treatment patterns
- Cost of treatment and side effects

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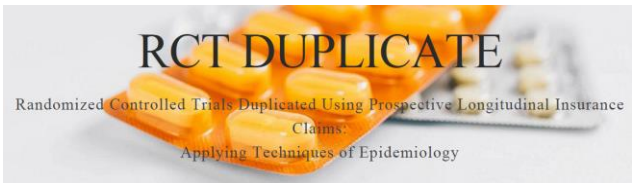
Obsession with using RWE to replicate RCTs

MULTI-REGIONAL CLINICAL TRIALS
THE MRCT CENTER of BRIGHAM AND WOMEN'S HOSPITAL and HARVARD

OPTUM Labs®

Observational Patient Evidence for Regulatory Approval and uNderstanding Disease (OPERAND)



JAMA Network | Open

Original Investigation | Statistics and Research Methods

Feasibility of Using Real-World Data to Replicate Clinical Trial Evidence

Victoria L. Bartlett, BA; Sanket S. Dhruva, MD, MHS; Nilay D. Shah, PhD; Patrick Ryan, PhD; Joseph S. Ross, MD, MHS

Cochrane Library
Cochrane Database of Systematic Reviews

Healthcare outcomes assessed with observational study designs compared with those assessed in randomized trials (Review)

Anglemyer A, Horvath HT, Bero L

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Live Content Slide

When playing as a slideshow, this slide will display live content

Poll: Can you infer causal effects from RWE studies