Innovative Clinical Development Solutions

Closing The Real-World Evidence Gap: Pragmatic Clinical Trials & Observational Studies

December 4, 2019

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Agenda

- Opening remarks
- Individual panelists presentations
- Moderated discussion with audience
  - Q&A dialogue
  - Audience to share experiences

Definitions

- **Real-World Data (RWD):** data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources.
- **Real-World Evidence (RWE):** clinical evidence regarding the usage and potential benefits or risks of a medical product derived from analysis of RWD.
- **Pragmatic Randomized Clinical Trial:** a randomized clinical trial (RCT) embedded into real clinical practice with eligibility criteria designed to enroll a diverse/broad population of patients and capturing clinical data already collected as a part of routine care.

Definitions - Continued

- **Patient Registry:** an organized system that uses observational study methods to collect uniform data (clinical and other) to evaluate specified outcomes for a population defined either by diagnosis of a disease (disease registry) or usage of a treatment (exposure registry).
- **Prospective Observational Study:** a non-interventional clinical study in which the population of interest is identified at the start of the study, and exposure/treatment and outcome data are collected from that point forward.
- **Retrospective Observational Study:** a clinical study that identifies the population and determines the exposure/treatment from historical data (i.e., data generated before the initiation of the study).

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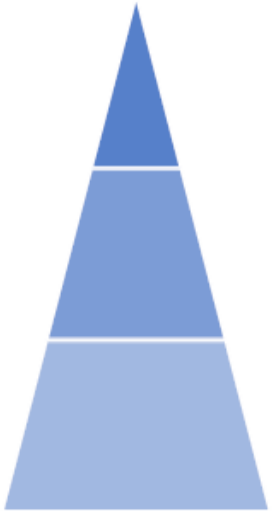
Explanatory & Pragmatic RCTs & Observational Studies

	Interventional?	Randomized?	Prospective?	RWD Collection
Explanatory RCT	Yes	Yes	Yes	
Pragmatic RCT	Yes	Yes	Yes	
Single arm trial with external control	Yes	No	Yes	
Patient Registry	No	No	Yes	
Prospective Observational Study	No	No	Yes	
Retrospective Observational Study	No	No	No	

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Speakers

Robert M. Califf, MD, MACC

- Vice Chancellor for Clinical and Translational Research, Duke University

Jane Liang White, ScD

- Sr. Director, Statistical Group Lead for Oncology Hematology Franchise, Pfizer

Rebecca Miksad, MD

- Senior Medical Director, Flatiron Health

Miganush Stepanians, PhD

- President and CEO, PROMETRIKA, LLC (Moderator)

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Overview of Discussion Topics

- Real-World Evidence in Drug Development
  - Regulatory Perspective
- Pragmatic Clinical Trials and Observational Studies
  - Present and Future
- Case Study: Single Arm Trial with Synthetic Control Arm
  - Statistical and Study Design Considerations
- Sources of Real-World Data
  - Available Databases

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