Guide for Real-World Evidence

Real-world data (RWD) from electronic medical records, insurance claims, and disease registries have expanded what Biotechs can learn about patient outcomes. Turned into real-world evidence (RWE), this is fast becoming a go-to tool for Biotech companies to optimize asset valuation, inform strategy and support regulator and payer decisions.

Parexel Biotech has created this guide to simplify the nomenclature and types of real-world studies.

Acronyms explained

Real-World Data (RWD)

Data relating to patient health status and/or the delivery of health care is routinely collected from various sources, including patient registries, electronic medical records, claims databases, or even from wearables and other medical devices. The data must be interpreted appropriately for application in clinical, policy, or other decision-making.

Real-World Evidence (RWE)

Evidence describing clinical evidence about the usage and potential benefits, or risks of a medical product, or practice. RWE is derived from the transformation, analysis, and interpretation of RWD for use in clinical, policy, payer, or other decisions.

Real-World Study (RWS)

See Observational Study

- NB: no global harmonization yet exists on nomenclature. E.g.,
  - FDA uses term, 'observational epidemiologic study' in guidance
  - EMA uses non-interventional (NI) study in legislation and guidance
  - China uses RWS in RWD guidance, further clarifying they are observational studies

Study types

Retrospective observational study

An observational study that identifies the population and determines the exposure/treatment from historical data (i.e., data generated prior to the initiation of the study). Variables and outcomes of interest are determined at the time the study is designed. Some examples may include retrospective chart reviews or analyses of historical data already existing in other sources.

Prospective observational study

An observational study that identifies the population of interest at the start of the study, and exposure/treatment and outcome data are collected prospectively and without intervention from that point. The start of the study is defined as the time at which the research protocol for the specific study question is initiated.

Hybrid real-world study

A study incorporating both primary data collected specifically for this study (typically prospectively) and secondary data within the same study. Secondary data include data that already exist for another purpose – such as medical records, claims databases, or existing databases/registries – but will be repurposed for use in this study.

Natural history study

A natural history study is a preplanned observational study intended to track the course of a disease, where individuals are followed from disease onset until disease resolution or death.

External vs synthetic controls

External Control Arm (ECA)

- A study comparator arm consisting of patients who are not part of the same randomized study as the group receiving the investigational agent (i.e., no concurrently randomized control group). The comparator arm is "external" to the investigational trial.

Historical external control

- A population of patients serving as the external control observed at an earlier time as the trial.

Contemporaneous external control

- A population of patients serving as the external control, observed at the same time as the trial.

Synthetic control

- A population of patients created as a control arm where patients are drawn from one or more external data sources and aggregated or "synthesized" into a comparator arm. This is one subset of ECAs; other ECAs may include patients that already existed as the control arm to another trial.

Driving innovation

As the use of real-world evidence (RWE) accelerates because it supports faster, more cost-effective drug and vaccine development, the next frontier is using it to demonstrate efficacy. Parexel's recent pioneering efforts in external control arms have enabled companies to be more effective at collecting real-world data (RWD) and generating RWE to support regulatory and HTA submissions.

To learn more, **download our Oncology eBook** or connect with one of our experts today.

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