VERISTAT

How Does RWE Impact Clinical Trials?

The use of Real-World Evidence (RWE) across the product development cycle is growing, gaining increasing attention and acceptance from regulatory agencies.

What Is Real-World Evidence?

RWE is defined as the evidence gained from the analysis of Real-World Data (RWD). RWD is data obtained from multiple sources, including electronic health (medical) records and patient registries. It can also come from health applications and environmental data, among sources other than clinical trials.

Sponsors conducting clinical trials for rare disease therapies often face the challenge of small patient populations, limiting statistical power. Patient healthcare data collected outside of a clinical trial can help paint a clearer picture of a novel therapy's benefits and risks.

RWE/RWD is intended to:

- Provide a better understanding of a therapy's safety and effectiveness in the real-world.
- Deliver insights into the therapy's performance in a population broader than in a clinical trial.
- Identify patient subgroups that may benefit from the therapy or who may be at increased risk of adverse events.

Did you know that the global healthcare sector was estimated to have generated 2.3 zettabytes of data by 2020—the equivalent of 2.3 trillion DVDs?

What Are Some of the Challenges of Using RWD?

FIT FOR PURPOSE
RWD can be incomplete, inaccurate, or inconsistent, impacting the relevance and reliability of analysis.

DATA INTEGRATION
Combining data from multiple sources can be problematic.

BIAS
Patients may not be representative of the overall population.

STATISTICAL COMPLEXITY
Analyzing RWD often requires sophisticated statistical methods to properly interpret results.

Statistical methods are available to help meet these challenges.

Are Natural History Studies Considered RWD?

Natural history studies are a type of RWD because they collect observational data from patients. They provide insights into the natural progression of a disease without experimental interventions, often utilizing sources like disease registries to capture how a disease unfolds in the real world.

NATURAL HISTORY STUDIES CAN HELP

1. **INFORM clinical trial design**
2. **CHARACTERIZE disease progression in untreated patients and confirm key inclusion/exclusion criteria used in clinical trials**
3. **SERVE as a comparator group when evaluating new therapies**
4. **IDENTIFY or develop clinical outcome assessments and biomarkers**

Where Do Regulatory Authorities Stand on the Use of RWE and RWD?

The use of RWE/RWD is modest but growing, with a limited contribution to the decision-making process. Regulatory authorities are continuing to further clarify and define guidelines and frameworks for using RWE and RWD in regulatory decision-making, including pre-marketing, marketing authorization, post-marketing studies, and label extensions.

- FDA has a long history of using RWD and RWE to monitor the post-market safety of approved drugs.
- RWE has also been used to support therapy effectiveness in clinical trials, but on a more limited basis.
- FDA recently announced the new Center for Real-World Evidence Innovation (CCRI) to advance the use of RWD and RWE in regulatory decision-making across the Center for Drug Evaluation and Research (CDER).
- The EMA supports RWE through initiatives like the Data Analysis and Real World Interrogation Network (DARWIN EU), aiming to provide reliable evidence on medicinal products' use, safety, and efficacy.

How Has Veristat Successfully Leveraged RWE and RWD?

Veristat works closely with sponsors to identify and collect applicable RWE and RWD through:

- **Prospective and retrospective natural history studies**
- **Accessing registry data that can be matched to clinical trial cohorts**
- **Accessing and analyzing medical claims data**

RESOURCE LINKS

VIDEO
RWE/RWD: Takeaways from FDA's Dec 2024 Webinar >

BLOG
How does Real-World Evidence Impact Medicinal Product Development in Europe? >

PRESENTATION
The Role of Natural History Studies in Ultra-Rare Disease Trials >

Learn more

With our integrated statistical, data management, consulting, and regulatory services, and first-hand experience communicating directly with FDA, Veristat can help you leverage RWD and RWE in a valid and compliant manner. Uncover insights to accelerate your therapy's success with the expert insights of Veristat.

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