Real-World Studies

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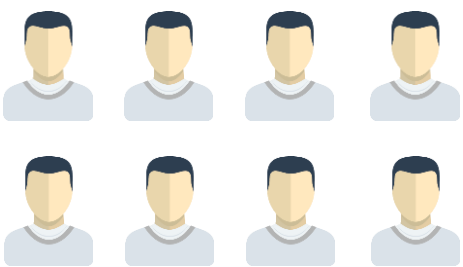
Randomized, controlled trials (RCTs) are used to evaluate treatment outcomes.[1] An important, complementary source of knowledge is "real-world" data.

Real-world data contribute to our understanding of treatment effectiveness and safety, disease and treatment patterns, and patient behaviors in everyday clinical practice.[2-4] These data are collected outside of the controlled environment of conventional RCTs.[2-4]

CLINICAL TRIALS AND THE REAL WORLD

Most clinical trials are strictly controlled with specific inclusion and exclusion criteria and investigate carefully defined patient populations.



In clinical trials, inclusion and exclusion criteria for the targeted patient population may influence outcomes.[5] They excel at answering scientific questions about how well a treatment works and its potential side effects in controlled conditions. Participants are often randomly assigned to different groups in the study, which is the most effective means of minimizing biases that could lead to false conclusions.[5]

The patients who receive treatment in the real world can differ in important ways from the patients enrolled in the clinical trials for that treatment.[6] Real-world patients may be older, have more medical conditions, and have more advanced disease.[6]

ANOTHER SOURCE OF INSIGHT

Real-world studies can help us better understand how a treatment is used in everyday clinical practice. By studying how a treatment is prescribed by health care providers and used by a diverse patient population in everyday environments, we gain insights about the people and settings underrepresented in RCTs. Real-world studies may:

- Lead to improvements in the way medicines are prescribed and used among these patient populations.[2]
- Uncover rare side effects that are difficult to detect with an RCT.[3]
- Examine health economics issues, such as healthcare resource utilization and costs.[2]
- Generate hypotheses to be tested in future RCTs.[2]

CONSIDERATIONS FOR REAL-WORLD DATA

In capturing the reality of the real world, we have to rely on applying validated methodologies to assess and interpret imperfect data.[6]

EXAMPLE

A database of heart disease patients may contain thousands of patients, but some may be missing information about the dose of over-the-counter medicines received. Perhaps the way the disease was diagnosed was inconsistent across patients, or tests were performed improperly. We must carefully consider these potential sources of bias.

Real-world data must be interpreted thoughtfully, keeping in mind the various ways in which it was collected and analyzed. Gathering real-world data is a serious undertaking that should be conducted with the same rigor and intent as an RCT. It is not a matter of just pulling numbers from a database or sending out a quick survey.[6]

DIVERSE REAL-WORLD DATA

Real-world data can come from many sources. It includes prospective observational studies designed to collect data on real-world patients. It can also retrospectively draw on existing patient registries, insurance databases, and electronic medical records.[4] There is a lot of room for innovation in collecting real-world data. As with RCTs, real-world data must be collected with careful consideration for ethics and patient privacy.

COMPLEMENTING, NOT REPLACING CLINICAL TRIALS

Used together, both real-world studies and RCTs contribute to the understanding of a treatment or disease. They are complementary, rather than substitutes for each other, because they provide data from different settings.

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