Emulation of randomized clinical trials with nonrandomized database analyses

April 26 2023

Credit: Pixabay/CC0 Public Domain

The complete findings of the RCT-DUPLICATE demonstration project are published today in *JAMA*.

This three-year initiative tested whether researchers asking clinical questions in real-world data (RWD)—data from patients' day-to-day interactions with the health care system—would obtain similar results to findings from randomized clinical trials (RCTs). In cases where clinical trial designs aligned with real patient care processes, the RWE studies and RCTs came to largely similar conclusions.

As part of the RCT-DUPLICATE initiative, researchers created and published protocols for observational, real-world analogs to thirty completed and two ongoing RCTs, emulating each trial's design as closely as possible.

They then implemented the studies principally using the Aetion Evidence Platform (AEP), which analyzes data from the real world to produce transparent, rapid, and scientifically validated answers on the safety, effectiveness, and value of medical treatments.

The results show that in about half of the cases where researchers were able to closely mimic the design of the corresponding RCT using RWD, the RWE study came to a similar conclusion as the analogous RCT. In many cases where RWE and RCTs did not come to a similar conclusion, the RCT design itself did not align with real-world clinical practice, creating a challenge for emulation of the trial using RWD. In these instances, RWE and RCTs may both be reaching meaningful conclusions, but to subtly different research questions.

"The RCT-DUPLICATE initiative further demonstrates that RWE has the potential to augment findings from RCTs and guides us to cases where RWE and RCTs may be expected to reach similar conclusions," said Nicolle Gatto, Ph.D., M.P.H., Chief Science Officer at Aetion.

"This work is important in promoting and understanding the value of RWE for decision-making as we continue to assess RWE's uses, benefits, and limitations."

More information: Shirley V. Wang et al, Emulation of Randomized Clinical Trials With Nonrandomized Database Analyses, *JAMA* (2023). DOI: 10.1001/jama.2023.4221

Provided by Aetion