

ALL FOR ONE

The N-of-1 trial design aims to provide a definitive answer as to whether a treatment works in a particular patient. As such, the entire process of testing a treatment is personalized to that patient—from the selection of measurable outcomes to the use of data once the trial is over. The approach therefore differs from most randomized controlled trials (RCTs), which are usually geared toward answering a particular research question. Yet despite their individualized design, N-of-1 trials can also be useful in clinical research. Data collected from multiple N-of-1 trials can be aggregated and—provided that the correct statistical tools are applied—analyzed to generate population-level data about drug response, while capturing far more information about intra- and interindividual heterogeneity than most RCT designs.

TYPICAL N-OF-1 TRIAL



STANDARD RANDOMIZED CONTROLLED TRIAL (RCT)

A patient works with her physician to develop a study design with the primary goal of finding the best course of treatment for her.



A patient signs on to a clinical trial with protocols and outcome measures determined by clinicians with the primary goal of answering a research question about the intervention.



She alternates between taking a drug and a placebo during the course of the trial. The drug and placebo are made into identical capsules, which she takes in treatment blocks of several days or weeks at a time in a randomized sequence.



Although some designs—for example, crossover trials—involve multiple treatment periods, a patient in an RCT will often take either a drug or a placebo for the entire duration of the trial.



The patient receives detailed feedback at the end of the study about her results, plus a recommended treatment plan based on that information.



Patients typically do not receive individualized feedback about the trial, and only in some cases are they notified when study results are published—if they are published at all.



Provided patients consent to be involved in research, clinicians can use special analytical techniques such as Bayesian statistics to aggregate data from multiple N-of-1 trials in order to make inferences about a particular therapy's efficacy at the population level. These techniques account for the fact that trials were carried out individually, and capture information about inter- and intraindividual variation in drug response.



Researchers analyze group-level data from the trial to make general statements about the drug's safety and efficacy for the population tested. Only limited information about interindividual variation is available.

