

N of 1 trial

When to conduct N-of-1 trial?

- The clinician is uncertain whether a treatment that has not yet been started will work in a particular patient.
- The patient has started taking a medication, but neither patient nor clinician is confident that a treatment is really providing benefit.
- Neither the clinician nor the patient is confident of the optimal dose of a medication the patient is receiving or should receive.
- A patient has symptoms that both the clinician and the patient suspect—but are not certain—are caused by the adverse effects of the medications.
- The patient has so far insisted on taking a treatment that the clinician believes is useless or harmful, and although logically constructed arguments have not persuaded a patient, a negative result of an n-of-1 RCT might.

When is N-of-1 RCT feasible in a patient?

- Patient is eager to collaborate in designing and carrying out an n-of-1 RCT
- The treatment has rapid onset and termination of action; the underlying condition must not be modified by the treatment – only symptom control must be possible.

- An optimal duration of treatment is feasible
- Patient-important targets of treatment are amenable to measurement
- The criteria to end the n-of-1 RCT are identifiable
- There is a pharmacist who can help with blinding
- There are strategies in place for gathering and interpretation of the outcome

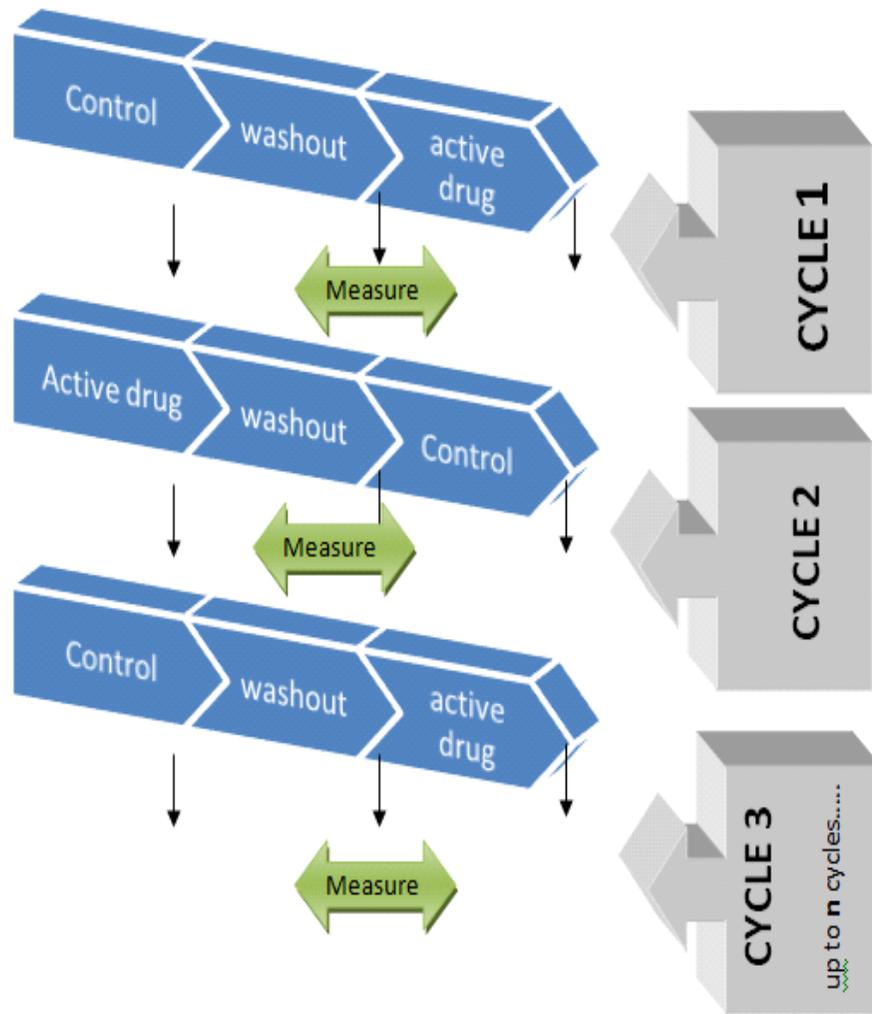
How to conduct N-of-1 trial?

Both patient and doctor remain blind. The pharmacist delivers control and active drug in identical form. The allocation in each cycle is randomly determined preferably by a third party.

Regular measurement of outcome is carried out. The patient and doctor agree when to stop the experiment. It is often done when a strong pattern has emerged with respect to the efficacy or lack of it. So there may be many cycles of treatment before it is ceased.

The data is then analysed to see which intervention fared better.

Guyatt G. et al. JAMA User's guide to medical literature, 2nd ed. McGrawHill Medicine 2007. Pg 180



Stats

SPMM Memory Corner

- N-of-1 trial has only one subject
- Generalisation of results to other patients is NOT possible
- Best EBM for answering therapy related question in a given patient, provided it is feasible