The language of research (part 15): research methodologies: randomised controlled trials (3)

KEY WORDS
- Bias
- Data collection
- Hypothesis
- Randomised
- Reliability
- Validity

PETER ELLIS
Independent Nursing and Health Care Consultant, Writer and Educator

In this paper part of the decoding science series, we will continue to explore randomised controlled trials (RCTs). In the first two papers in this three part mini-series, we identified RCTs as a methodology that is experimental in nature and that follows some strict rules to ensure validity and reliability are maintained and bias is avoided.

In reality, all research undertaken in human subjects is suboptimal in some way or another, but by managing some of the issues associated with study design and data collection, the quality of the research can be optimised. The best studies also acknowledge the compromises they made in undertaking the study in the discussions and conclusions of the study write up, so readers can make their own assessment of quality.

This paper will look at some of the issues concerning the methods used for the collection of data in RCTs and make some final comments about the pros and cons of undertaking RCTs. First of all we will look at the nature of how the research question is asked in RCTs so that we can understand the nature of data collection needed to address it.

THE NULL HYPOTHESIS

In paper one in this three part series, we stated that "Interventional studies seek to manipulate an exposure (the independent variable) in order to measure what effect it has on an outcome (the dependent variable (Gordis, 2014))". That is to say that in RCTs the research question, or the topic to be investigated, is usually framed in the manner of we are doing something because we expect it to cause a particular outcome. In this respect, experimental studies are deductive in that they seek to prove something, rather than allowing the evidence to emerge and generate an understanding as is the case in inductive studies (Ellis, 2016).

In experimental studies it is therefore usual to have a hypothesis. Hypothesis means something which is less than (hypo) a theory (thesis) because it has yet to be proved. In RCTs, it is more usual for the question to be posed as what is called a null hypothesis. A null hypothesis is in essence a hypothesis which states that there will be no difference in the outcomes between the treatment and the non-treatment arm in the study, or that there is no discernible association between variables (Greenwald, 2009).

The reasons for having a null hypothesis relate to the manner of proof in statistical testing and scientific analysis are not important here.

DATA COLLECTION METHODS

There are a number of methods used for collecting data for RCTs, these are dependent on a number of distinct, but interrelated issues:

- The topic being researched
- The outcome(s) of interest
- Issue of validity
- Issues of reliability
- Avoiding bias and confounding
- Practical issues
- Ethical issues.

One of the most important elements of the research design of an RCT is that it takes into account the need to collect data which is salient to the topic being researched and it does so accurately and is clear in the description as to how this is achieved (Gersch et al, 2010).

The single most important element of data collection in RCTs is the avoidance of bias; that is the systematic introduction of error to the research process (Ellis, 2016).

Data collection for RCTs may include clinical elements, which tend to be more objective, such as wound size, rates of healing, incidence of wound infection, etc. Other issues may also be measured, these include the patients' experience of pain, quality of life and the patients' perceptions of their care which all tend to be more subjective.

When assessing the quality of an RCT the readers should look at the aims and objectives, often contained within the null hypothesis, of the research and ensure that the tools used to collect data reflect these. Almost all RCTs now collect data on several outcomes. They will use clinical measures and non-clinical measures during the study to obtain data to answer the research question(s).

As mentioned previously, all clinical measures undertaken as part of data collection need to be valid (they measure what they state they measure) as well as reliable (they measure in a reproducible way). Clinical measures need to be undertaken objectively and as stated in paper two of this series (Ellis, 2017) they preferably need to be collected in such a way that the person collecting the data is blinded as to whether the participant is in the treatment or the control arm of the trial.

All clinical measures should be undertaken using the current agreed gold standard method wherever this is possible. Some of the clinical measures will use pre-validated tools (such as questionnaires) to measure the outcomes the researchers are interested in. Examples used in wound and pressure ulcer healing include:

- Pressure Ulcer Scale for Healing (PUSH) (National Pressure Ulcer Advisory Panel, 2007)
- Bates-Jensen Wound Assessment Tool (BWAT) (Sussman and Bates-Jensen, 2007)
- The Sessing Scale (SS) (Ferrell at el,1995)

These tools have been used on a variety of occasions to measure healing of wounds and ulcers and their levels of validity and reliability are well documented (Pillen et al, 2009).

Other quite common examples of validated questionnaires include the Short Form 36 (SF36), which is used to measure an individual's health and wellbeing (Rand, ND) and the Manchester Short Assessment of Quality of Life (MANSA) tool (Priebe et al, 1999), which uses 16 questions to assess the participants' quality of life.

Where researchers use their own tools to collect data, the content of the tool needs to be justified and issues around validity and reliability discussed.

PROS AND CONS OF RCTS

As with all study designs, there are both pros and cons to the use of RCTs, some of with are listed below. When reading a RCT, it is worth bearing these issues in mind when you are considering the adoption of the findings in your clinical practice.

Pros

- Proves a cause and effect relationship
- If long enough can show safety of an intervention
- If done well, randomisation is a good way to control issues such as confounding
- If blinding is done well, can minimise the occurrence of bias
- Because it is prospective, it is good at demonstrating the temporal sequence of events
- Can be used for high-quality statistical analyses and therefore is often open to use in meta-analyses of data
- Is able to measure both incidence of disease and also multiple outcomes if designed well and the right tools are put in place.

Cons

- RCTs, especially multi-centre ones, are very expensive to do and are time consuming
- The design of a good study can be very complex
- Analysis is often specialised
- Some proposed studies have inherent ethical difficulties and cannot be undertaken
- Rare outcomes are hard to study as are outcomes which take time to emerge
- Participants in research may not be representative of the general population and, therefore, findings may not be generalisable.

CONCLUSIONS

Within the last three papers in this series we have explored how RCTs are designed and why, we have explored some of the issues with managing data collection and quality. Most of all we have seen why RCTs are considered to be perhaps the most important study methodology used in healthcare and therefore why they are used to answer many of the key clinical questions.

We have seen that the quality of design and process are important in establishing the credentials of a study and that anyone reading a research report for an RCT should ask some questions about its quality rather than taking its finding at face value. WUK

REFERENCES

Ellis P (2016) *Understanding Research for Nursing Students (3rd edn).* Sage, London

Ellis P (2017) The language of research (part 15): research methodologies: randomised controlled trials 2. *Wounds UK* 13(4): 146–7

Ferrell BA, Artinian BM and Sessing D (1995) The Sessing scale for assessment of pressure ulcer healing. *J Am Geriatr Soc* 43 (1): 37–40

Gersch C, Macnee CL, McCabe S and Rebar C (2010) *Understanding Nursing Research: Reading and Using Research in Evidence Based Practice (3rd edn).* Wolters Kluwer/ Lippincott Williams & Wilkins, London

Gordis L (2014) *Epidemiology (5th edn).* Saunders, Philadelphia PA

Greenwald A (2009) Consequences of prejudice against the null hypothesis. In: Keren, G and Lewis C (eds.) *A handbook for Data Analysis in the Behavioral Sciences; Methodological Issues.* Psychology Press, New York

National Pressure Ulcer Advisory Panel (2007) *Pressure Ulcer Scale for Healing (PUSH) Tool.* Available at: http://www.npuap.org/resources/educational-and-clinical-resources/push-tool/ (accessed 11.12.2017)

Pillen H, Miller M, Thomas J et al (2009) Assessment of wound healing; validity, reliability and sensitivity of available instruments. *Wound Practice and Research* 17 (4): 208–17

Priebe S, Huxley P, Knight S, Evans S (1999) Application and results of the Manchester Short Assessment of Quality of Life (MANSA). *International Journal of Social Psychiatry* 45(1): 7–12

Rand Health (ND) *36-Item Short Form Survey (SF36).* Available at: https://www.rand.org/health/surveys_tools/mos/36-item-short-form.html (accessed 11.12.2007)

Sussman C, Bates-Jensen B (2007) *Wound Care. A Collaborative Practice Manual for Health Professionals (3rd edn).* Wolters Kluwer/ Lippincott Williams & Wilkins, Philidelphia