Clinical Trials IV Assignment 1

Friday 7th February 2025

In this assignment you are going to design, run and analyse a randomized controlled trial, and submit a report documenting your work. The report should be no more that 10 pages long (including any figures and tables, but not including title page, declarations, references etc.).

Practical details

This assignment is summative, worth 50% of your module mark. It is due in on **Monday 3rd March at 12 noon**. If you think you will need an extension, please contact the teaching and learning team (maths.teaching@durham.ac.uk).

Your submission should take the form of a report, which you can write in LaTeX, RMarkdown or MS Word (or similar), and submit via gradescope. You may have to convert a .docx file (or similar) to .pdf to submit it.

Running the trial

To generate your trial data, you will need to use a shiny dashboard, which you can find at

https://racheloughton.shinyapps.io/CT4_Assignment1_2425

You will need to keep interacting with the dashboard as you progress through the stages of your trial. I've tried to be as clear as possible, and to check that the dashboard works in as many ways as I can think of, but if you have problems that don't seem to be solved by carefully going over the instructions, or anything seems strange, please don't hesitate to contact me!

Trial Scenario

This trial involves patients of age 50-65 who suffer from a particular condition. One of the symptoms of this condition can be measured on a continuous scale. It is hoped that the treatment we're investigating **reduces** the extent of these symptoms. As eligible patients present at their GP surgery during the recruitment period, they will be invited to join the trial. Some personal data will be recorded, and they will each be invited back to the surgery to have a baseline measurement of the symptom taken later that week, following which they will immediately begin treatment (or placebo).

You can assume that the standard deviation of the symptom's value will be approximately 7 for both the baseline and outcome measurements and for both control and treatment groups, and that the mean of the baseline measurement is around 107. The participants' sex, age (ranging from 50-65) and BMI will also be recorded. The mean BMI measurement is expected to be around 27, with a standard deviation of around 4. You can also assume that the correlation between baseline and outcome measurements is around 0.7.

The clinicians would like to be able to detect an effect size of at least 2 (in the units of the symptom measurement), with a power of 85% and at a significance level of $\alpha = 0.05$.

Throughout your report, it's fine to refer to 'the condition', 'the symptom' etc. The vagueness of these terms is intended to stop you from importing any details about real conditions, since the data is likely not to be especially realistic!

Report

The report will be marked out of 50, with the divisions given below. There should be four main parts to the report:

1. Sample size (5 marks)
2. Allocation (13 marks)
3. Analysis of results (20 marks)
4. Trial considerations, explained below (12 marks)

In the fourth part, please identify three choices you have made in parts 1-3 and discuss their importance in relation to conducting an ethical and effective trial.

You must also include the plagiarism declaration and declaration of use of AI from the Projects IV module (hopefully you are all familiar with these - if not please ask). Please remember that this is an individual project, and instances of collusion or copying will be taken very seriously: in some cases we may hold a viva to verify genuine understanding.

What will this be marked on?

At all stages, I am looking for evidence that you are considering what we have covered in the first part of Clinical Trials IV, and are using that information to design and analyse the trial effectively and appropriately. This will show itself in the choices you make and your explanations of those choices, and in attention to detail in applying statistical methods and interpreting their results.

Try to imagine that these are real participants, and a real drug, that could benefit (or not) lots of people, depending on the results of this trial!

What am I not looking for?

There is no need to derive equations we have used in notes (whether or not I have derived them). There is also no need to include R code, unless you feel it demonstrates some significant insight or learning.

I am not looking for you to venture outside of the content of Clinical Trials IV (by which I mean the lecture notes, the formative assignments and the computer practicals).

I am not looking for one unique solution or path. You are likely to all have slightly different datasets, and there are different approaches that could be justified.

I am not overly concerned with the style in which you write up your report, except that it should be clearly written and well-explained.