Clinical Trials IV Assignment 2

Wednesday 19th March

Practical details

This assignment is summative, worth 50% of your module mark. It is due in on **Monday 5th May 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 RMarkdown, LaTeX, MS Word (or similar), and submit via gradescope. You may have to convert a .docx file to .pdf to submit it.

The Assignment

This assignment is organised very much like assignment 1, but with some slight differences. 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 etc.).

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_Assignment2_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, please don't hesitate to contact me!

Trial Scenario

This trial concerns patients who have recently been diagnosed with a particular, terminal condition. Patients who join the trial are assigned either to receive the standard form of care (group C) or to receive a new treatment (group T). The outcome variable is time until mortality, and patients are monitored for 9 months (274 days) after they join the trial.

It is thought that for patients in the control group a reasonable approximation to the time to mortality is given by the exponential distribution. The median survival time for the control group is around 103 days, and the trial clinicians would like to be able to detect an increase in median survival time of at least 35 days, with a power of 90% and at a significance level of $\alpha = 0.05$. They expect that around 5% of the participants will be lost to follow-up during the life time of the trial. The clinicians are particularly interested to learn about the effect of the intervention in those aged 70 and over.

For each participant, the following covariates will be recorded:

- Disease level (moderate or severe)
- Age - all participants will be over 50 years old.

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 - you must do this using the simulation method (13 marks)
2. Allocation (7 marks)
3. Analysis of results (16 marks)
4. Trial considerations, explained below (14 marks)

For part 4, suppose some members of the trial team disagreed (at the planning stage) with some aspects of the proposed trial design and instead suggested recording which patients were alive at some predetermined point (say, after exactly six months). Discuss the implications of this alternative plan on the design and analysis of the trial, and also on the practicalities.

You may notice that the allocation section is worth proportionally less than for the first assignment, even though it requires the same amount of work. This is because it doesn't draw on any new (since week 5) material.

You must also include the plagiarism declaration from the Projects IV module (this is found on the Projects Ultra page and also in a Clinical Trials announcement from 17th February), and you must not use any generative AI. 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 Clinical Trials IV module (particularly the second half), and are using that information to design and analyse the trial effectively. This will show itself in explanations of choices, and in attention to detail in applying statistical methods.

Try to imagine that these are real participants, and a real treatment, 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 or models 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; the main exercise is piecing the disparate parts together appropriately, but all the information you need should be in the course materials.

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