The good, the bad and the ugly: reflections from conducting a trial in social care

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Overview

- Using a motivating example of a trial evaluating hearing dogs, we will:
- Discuss the design of the study
- Some of the challenges that were faced along the way
  - The way in which the statistical perspective helped
- Reflections moving forwards
  - What worked and what didn’t!

How it all started

- I was contacted by colleagues in the Social Policy Research Unit in York to see if I would be interested in helping design a study to evaluate the impact of hearing dogs
- Sounds very interesting count me I said….that was in 2005…

Hearing Dogs for Deaf People

Y T U York Trials Unit Department of Health Sciences

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CHE Centre For Health Economics

Hearing dogs – what are they?

- People with hearing impairment, compared to the wider population, have higher rates of:
  - Social isolation, emotional distress, mental health difficulties and impaired quality of life
- Hearing Dogs for Deaf People is a charitable organisation creating and supporting partnerships between specially trained dogs and individuals with severe/profound hearing impairments
- Hearing dogs are trained to recognise, discern and alert a deaf person to a range of sounds related to carrying out:
  - Everyday life (e.g. cooker timers, alarm clocks)
  - Communicating with others (e.g. telephone/minicom calls, doorbell, their name being called)
  - Personal safety (e.g. smoke alarms, emergency vehicle sirens).
- Once a hearing dog partnership is confirmed, additional 'bespoke' sound work training is also undertaken:
  - Depending upon needs of the individual and the setting
- Hearing dogs live permanently with their partner and are with them on a 24 hour basis.
  - Hearing dogs are allowed into settings into which pet dogs are not permitted

Challenge #1

What study design could we use?

What study design could we use?

- We had a number of restrictions to work within:
  - Needed to ensure the design could fit within usual processes for creating Hearing Dog Partnerships
  - There is highly variable times taken to match an applicant with a dog
  - HDfDP's commits to create a partnership within **2-3 years** of application
  - Issues associated with evaluating complex, 'non-traditional' interventions
  - Undertaking research in and with a third sector organisation 'naïve' to research
- We discussed lots of options:
  - Observational study
  - Quasi experimental design
  - Randomised controlled trial
    - Doing trials for a living I was keen to do this if possible!
    - BUT a conventional RCT was obviously not going to work

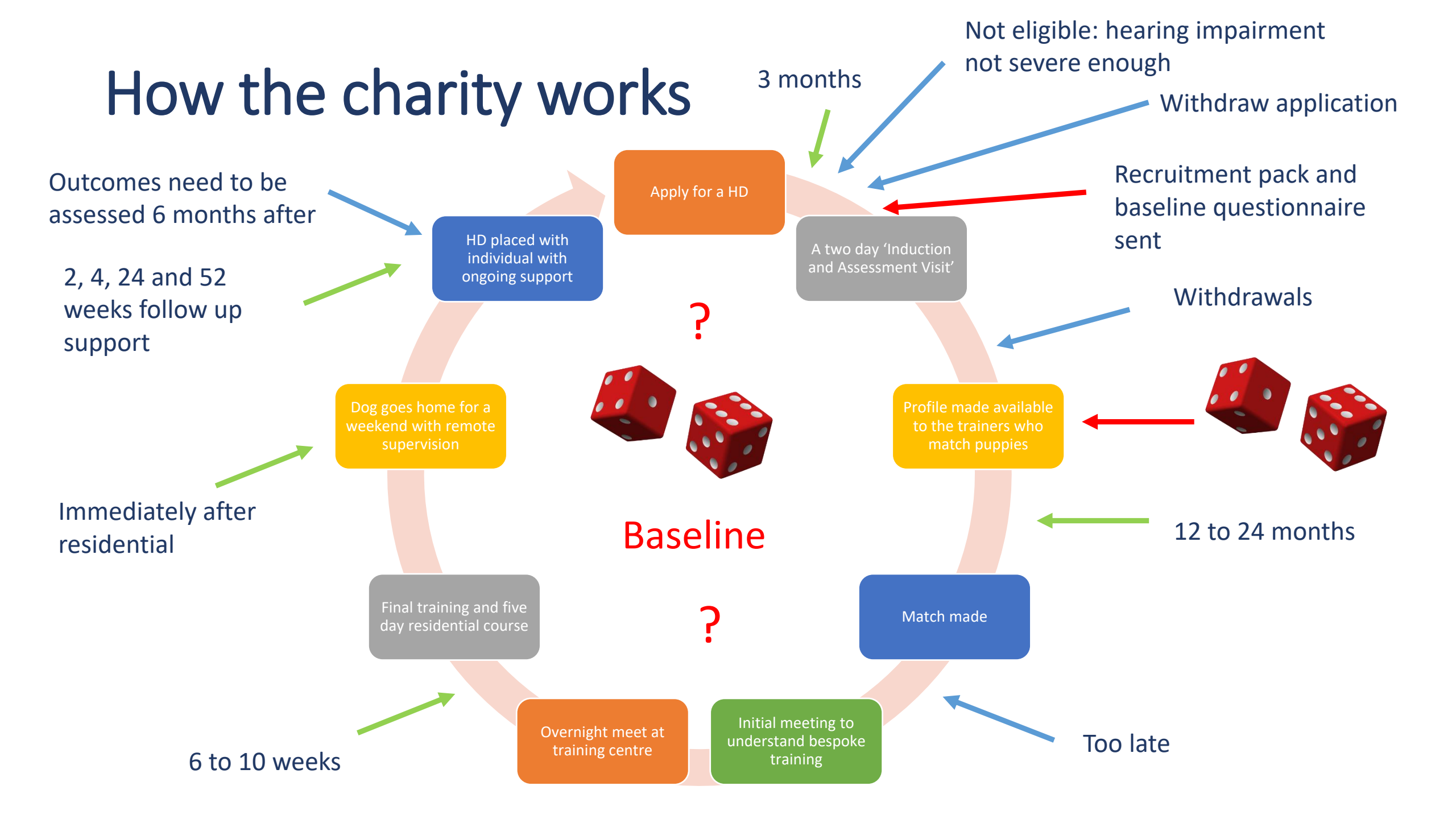
What study design could we use?

- Given that HDfDP's commits to create a partnership within **2-3 years** of application that got me thinking about using a waitlist type design
  - This would meet the charities requirement that no one would be waiting longer than usual
  - Would the charity let a "toss of a coin" decide who would receive their dog early or late?
- So the first challenge was solved by the creation of:
  - An accelerated vs usual application timeline
- This approach was also likely to be appealing to potential study participants who may receive their dog sooner than they might ordinarily have
  - Hence we hoped this approach would encourage participation
- Everyone who applied for a hearing dog during this period was invited to take part in the study

Challenge #2

When to randomise?

How the charity works



Challenge #3

How could we follow people up at the same time in the two groups when there was variable time to receive a HD?

Pairwise randomisation

- Usually in RCTs, we follow up all participants for a fixed period of time after the date of randomisation
- Whilst it is simple enough to ‘start the clock’ to follow up participants six months after they receive their hearing dog, the problem arises as to which control participant should be followed up to match the intervention participant
- We needed a trigger for the waitlist design when the control participants profile would be made available for matching after all participants had been followed up
- So the second challenge was solved by using pairwise randomisation
  - It allows us to identify, before the point of randomisation, a control participant that can be followed up at the same time point as the intervention patient and this avoids the potential of biased selection
  - You select a pair, one gets the intervention and one the control
- Pairwise randomisation was first described in 2003 but it is not often used in practice despite its simplicity

Challenge #4

How could we incorporate into the design and analysis the charities ability to override randomisation if needed? 😯

Non-compliance

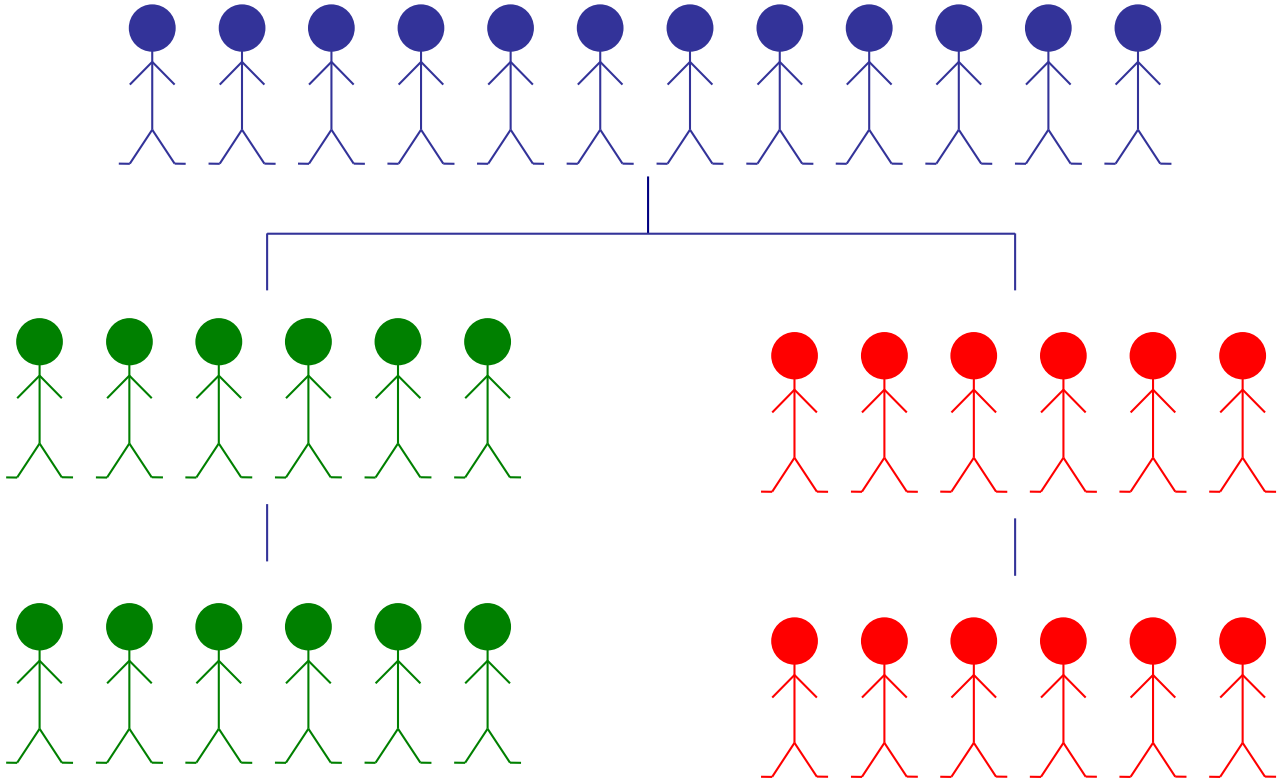
- In one of the meetings with the charity, they wanted to be able to allocate a hearing dog to a participant should the applicant's unusual/complex needs significantly reduce the number of hearing dogs with the required specific aptitudes/skills likely to become available within the 3 years HDfDP commits to applicants receiving a hearing dog.

Perfect trial participants

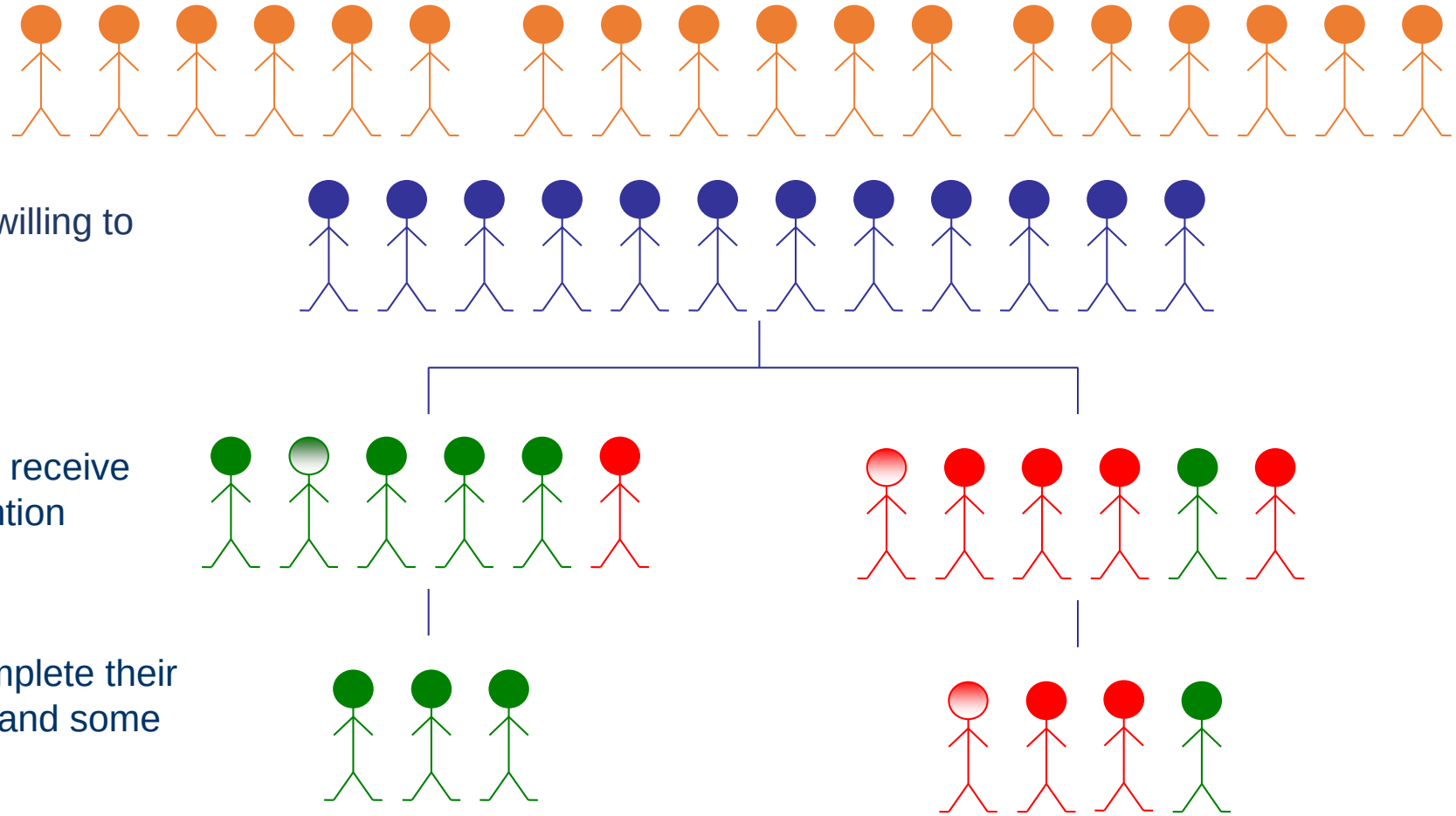
Everyone asked is willing to take part

Everyone recruited is randomised and receives their allocated intervention

Everyone randomised completes their allocated intervention and provides outcome data



What was going to happen here?



Some people are not willing to take part

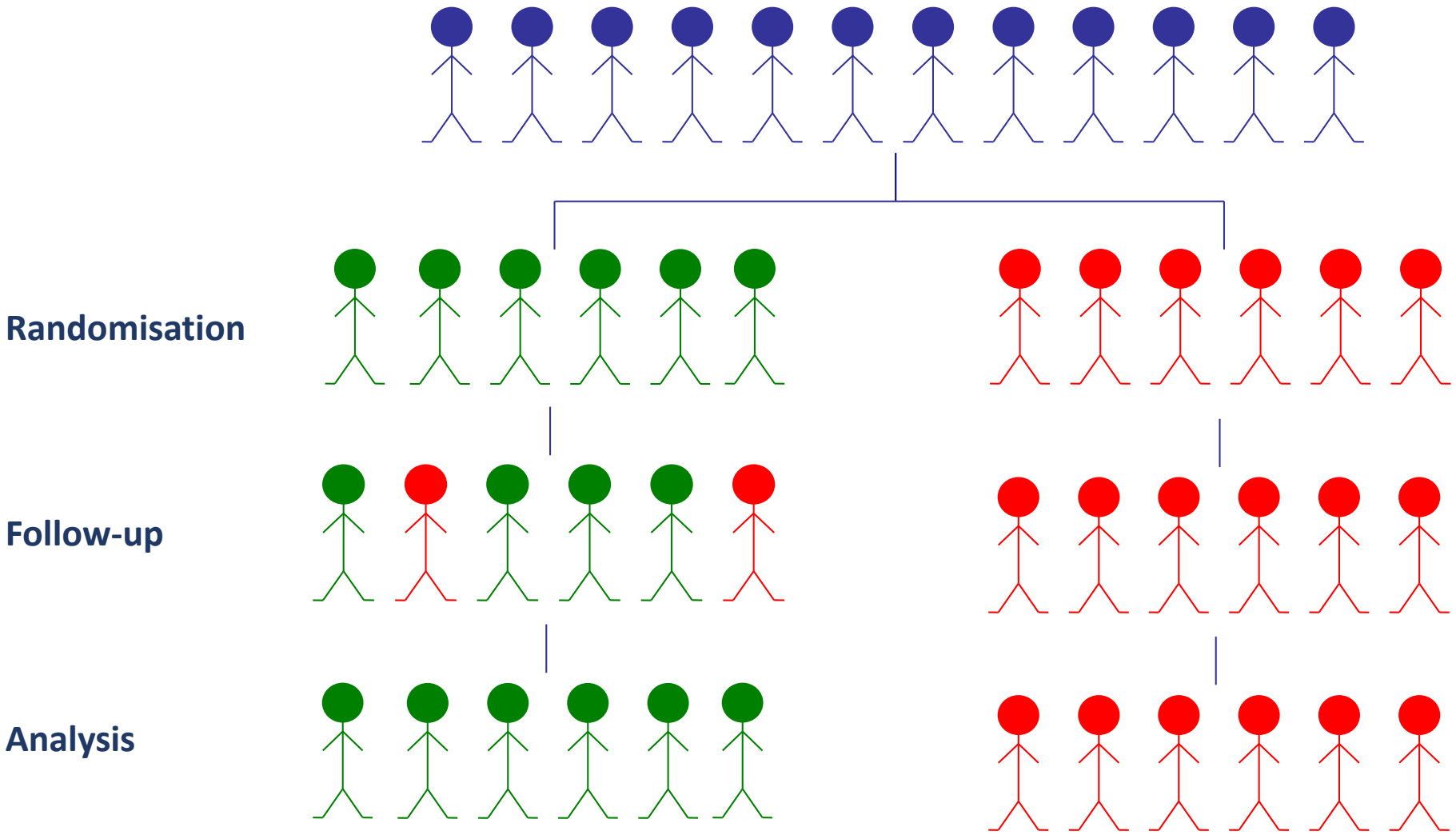
Everyone recruited is randomised and some receive their allocated intervention

Some participants complete their allocated intervention and some provide outcome data

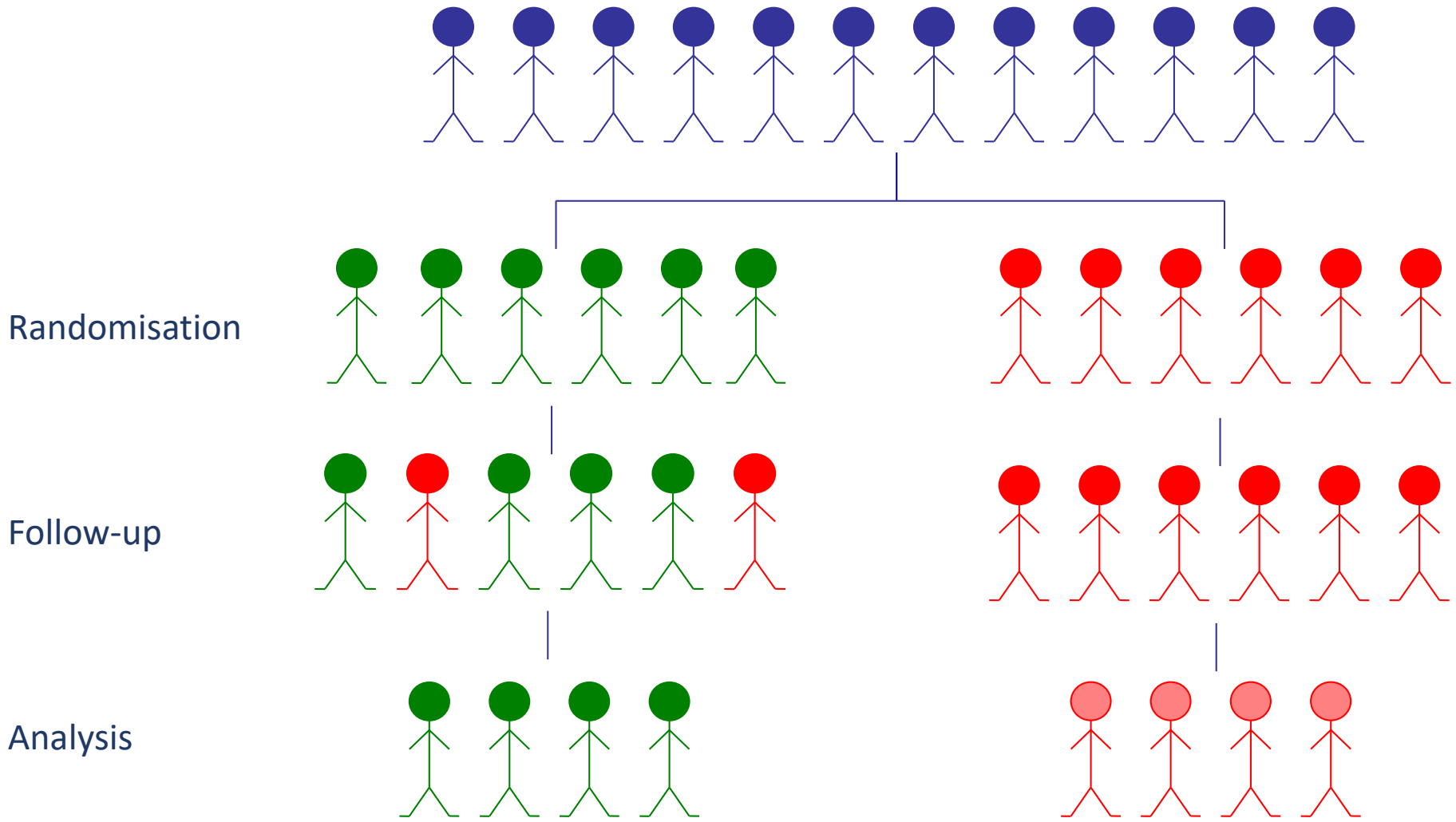
Problems during analysis

- Even in well designed and conducted RCTs non-compliance is almost inevitable
- Here the charity told us they needed this option!
- Explained the impact:
  - Creates problems at analysis stage
    - How should we deal with participants who do not comply with their allocated treatment in the analysis?
    - That it would dilute the treatment effect, so it would be harder to explore the impact of the intervention
    - BUT we needed to make things work so….

Intention To Treat



CACE: Complier Average Casual Effect



CACE

- General problem:
  - Need to identify the compliance status of the alternative group
- If this is possible, then
  - Compare the compliant group in the one group with the potential compliant group in the other group
- We need some measure of the compliance status in one arm
  - So as long as the charity records when everyone receives their dog and we can access this then that would work!
- Need to make some assumptions:
  - If the other group offered the intervention same proportion would not comply
    - Should be true due to randomisation
  - Being offered the treatment has no effect on outcomes
    - This sometimes needs more thought!

Challenge #5

Some people would return the dog, stop participating or withdraw?

Attrition bias

- Ideally we want to collect outcome data from everyone, irrespective of whether they receive the HD in relevant time period but that rarely happens
- Attrition can introduce bias if the characteristics of people lost to follow-up differ between the randomised groups
  - If differing characteristics are correlated with the outcome measure
- It is usual in trials to present baseline characteristics by trial arm
- Of more interest here is the comparison of baseline characteristics for those who remain in the trial
- Hence to explore the impact we planned to present baseline data descriptively by trial arm as randomised and as included in the primary analysis (i.e. baseline data for anyone with outcome data)
- If there were changes in balance in the ‘as analysed’ sample then that might indicate informative missingness and hence the need to explore this further in sensitivity analysis

Summary

- Challenge 1: What study design could we use?
  - RCT – Waitlist design
- Challenge 2: When to randomise?
  - Understanding the pathways
  - Randomise as late as possible to minimise dropout
- Challenge 3: How could we follow people up at the same time in the two groups when there was variable time to receive a HD?
  - Pairwise randomisation
- Challenge 4: How could we incorporate into the design and analysis the charities ability to override randomisation if needed?
  - ITT and CACE
- Challenge 5: Some people would return the dog or withdraw? Impact?
  - As randomised and as analysed comparisons
  - Missing data mechanism -> informative missing

Reflections

- Eligibility (n=279) and consenting and completing baseline (n=213, 76%) went well
- Before randomisation:
  - Withdrew application to HDfDP (n=40); other type of assistance dog needed (n=4); application paused (n=1); HDfDP withdrew (n=2); participant did not want to take part later (n=1)
- 165/279 (59%) eligible; 165/213 (77%) eligible and consenting randomised
  - Recruitment was slower than anticipated
- 112/165 (68%) followed up
  - Several participants did not receive a hearing dog within the data collection period so there was higher attrition than anticipated
  - Should have allowed more time for participants to receive HDs
- Things that helped:
  - Good working relationship with the charity throughout
  - Managing expectations
  - Study support office embedded within the charity
  - Methodologists involved in the design of the study

Finally

- Despite at the beginning there were people who said we could not do a trial in this complex area, we did do the trial!
- It is the first RCT to evaluate the impact of HD and their cost-effectiveness

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