NHS SCOTLAND
NHS RESEARCH SCOTLAND
NEUROPROGRESSIVE AND DEMENTIA

CLINICAL TRIALS FOR PARKINSON'S: A COMPANION GUIDE

Co-created by people living with Parkinson's for people with Parkinson's who are interested in clinical trials

Welcome

NHS SCOTLAND
NHS RESEARCH SCOTLAND
NEUROPROGRESSIVE AND DEMENTIA

Hello,

I'm Rosie and I lead Partners in Research, the 'patient and public involvement' group for NHS Research Scotland Neuroprogressive and Dementia Network (often shortened to NDN).

The NDN covers health boards across Scotland, and supports people with neuroprogressive conditions to get involved in research studies.

Within Partners in Research, we involve people with lived experience in multiple ways including:

LEARN MORE

SCAN HERE

- reviewing research and providing feedback
- promoting research participation and accessible research content
- designing and developing research that's meaningful to people with lived experience.

Together we have worked alongside people with experience of living with, or supporting someone with, Parkinson's to create this resource to help others navigate clinical trials research.

Our team includes Garry Sloan, Stephen Brannan, Joanna Goodburn and Marc van Grieken, who all have experience of Parkinson's and have been part of this project from the beginning.

We hope that you find it helpful. If you have any questions or suggestions, please get in touch.

tay.ppipartners@nhs.scot.

Dr Rosie Ashworth

Patient and Public Involvement Lead

Meet others with lived experience

Create research together

Give researchers feedback on their work

Summary

Our guide will walk you through the different types of research, and how we came to focus on clinical trials. We will share our experience of co-creating this resource for other people affected by Parkinson's, before exploring the reasons clinical trials may include or exclude someone from taking part.
Our online version of this resource also includes information about ongoing clinical trials, frequently asked questions and recommended resources.

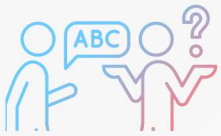
Key Learning from our guide:

1. **Clinical Trials are essential for progress:** They are the only way to find better treatments–and possibly a cure–for Parkinson's. Every participant plays a vital role, even if they don't directly benefit from the treatment being tested.

2. **Participation is voluntary, personal and supported:** Joining a trial is your choice. You'll be fully informed, carefully screened, and you can leave at any time. Research staff are there to support you throughout.

3. **Strict criteria exist to keep participants safe and ensure meaningful results:** Not everyone can take part, and that can be difficult to hear. Trials have detailed rules to protect participants and make sure the data collected is useful.

Glossary



Research has a tendency to come with a whole host of new words and jargon. We have created a list of definitions for some of the more unfamiliar terms or acronyms to help as you work through the guide.

- **Academia** refers to universities and research institutions where experts study, teach, and carry out research to expand knowledge and find new solutions to real-world problems.
- **Adverse Events** are unwanted or harmful effects that could happen in a clinical trial but may not be caused by the treatment
- **Biomarkers** are measurable signs in the body that researchers can test
- **Blinded** means that the research team are not told whether someone is on a new treatment/intervention or in the placebo/control group. They do this to reduce any bias in how the team report how someone is getting on in the study. If safety is a concern, a participant can be 'unblinded' so that the team know what they have been taking.
- **Co-research** refers to people with lived experience working alongside researchers to complete research together (co-research is done with people with lived experience rather than on people with lived experience)
- **CPN** is a Community Psychiatric Nurse
- **CRN** is a Clinical Research Nurse
- **CSO** refers to Clinical Studies Officer' who are part of the research team that work closely with people with lived experience and the principal investigator to carry out the study.
- **Data Integrity** makes sure the information that comes out of a clinical trial is accurate, trustworthy and can be relied upon for making decisions
- **Exclusion Criteria** is a checklist of characteristics that would make the research unsuitable for someone and so prevents them taking part in a clinical trial

Glossary

- **Inclusion Criteria** is a check-ist of characteristics researchers use to see whether someone is the best fit for a clinical trial
- **Phase 3 trials** tests whether a new treatment works better than current options in a large group of people, while closely monitoring safety and side effects.
- **PI or Principal Investigator** is the person who is responsible for the clinical trial at a local research site.
- **Placebo** in clinical trial research looks the same as the medication being tested, but does not contain the 'active ingredient' i.e. saline solution, sugar pills etc. They are used to see whether any benefit comes from the act of taking the medication and not the medication itself.
- **Pre-screening** is where a research team review potential participants for a research study based on a list of characteristics the study has provided them with.
- **Randomisation** is the stage in a study where participants are grouped for the remainder of the study e.g. control group(s) and intervention group(s).
- **Randomised Controlled Trial** is a type of research where people are randomly grouped into either receiving the intervention/study drug or a placebo/standard care. They are seen as the 'gold standard' of research design for comparing new treatments and interventions.
- **Research Safety** makes sure everything in a clinical trial is carefully planned and reviewed to protect your health and well-being at every stage.
- **Screening** is where potential participants work with the research team to see if the study is a suitable fit for them
- **Serious Adverse Events** are a more serious event that could result in hospitalisation or similar and can lead to a trial stopping or being changed.

Different types of research methods

- Research can look at large groups of people with a condition and observe how this evolves over time
- Other research asks people to share their experience at a specific time point in detail.

"I welcome talking about Parkinson's because I feel it is very important for people to get help and advice from in particular someone who has had experience.- ***Henry Muchamore***

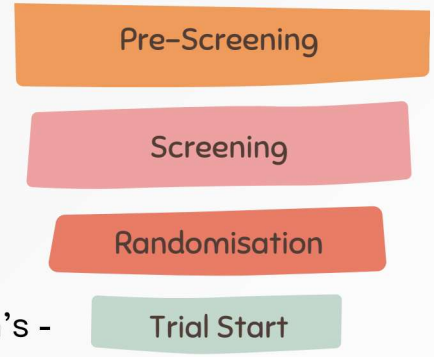
It's worth doing research as hope that the person asking the questions will get the answers for future generations.- ***Alan Miller***

- Researchers look for gaps in knowledge, new information or new approaches; and researchers will have their own preferences and expertise.
- Researchers often work together so that you have a mix of expertise looking at the same topic.
- Co-research is where people with lived experience design and develop the research alongside researchers. Co-research can widen our understanding of health conditions by making sure that research is relevant and meaningful to people with lived experience.
- The different types of research will all tell us different things about the topic being explored. One type of research is not better than another, it is simply what is the best fit for the question being asked i.e. an observational study using interviews is not going to cure Parkinson's, however, it could help us learn more about why people may not take part in research and how we can encourage others to do so in the future.

What is a clinical trial?

Research is the only way we can find a cure, new treatments and alternatives to current approaches.

- The NDN supports many clinical trials for Parkinson's, and details of which studies are going on in your area can be found online.
- Most commonly, clinical trials are **'randomised controlled trials'** which mean a group or groups of people are being 'tested' with something new e.g. a drug, and a group of people continue with routine care (which is known as the **placebo** group).
- Getting started on a clinical trial involves several steps:
  - **pre-screening** is the first step where staff consider if you might be eligible (e.g. if the clinical trial looks at low blood pressure and Parkinson's - do you have both of those two things?)
  - **screening** (which uses more in-depth tests e.g. brain imaging scans, questionnaires and medical history to see if you meet a pre-determined list of criteria set out in the research).
  - **randomisation** which decides whether you will be in the test group(s) or the control group.
- Clinical trial staff are there to make sure you understand what the trial would involve and whether it is a good fit for you. Staff are often **'blinded'** to the intervention i.e. they won't know if you are receiving the treatment or the placebo. This is to avoid staff unconsciously biasing their answers to how you are getting on in the trial.



"Clinical trials are really exciting. Many scientists around the world are getting close to finding, not only symptom alleviating treatments, but possibly even a cure. None of this work will lead to a drug you can take until enough trialling of those drugs has been done so I would really encourage anyone who does have Parkinson's to think about getting involved in these trials and helping us to find a cure.

As a person with a partner who signed up to be involved in a trial, I do realise it can be quite nerve wracking, there are risks involved, although by the time you get to a ***phase 3 trial****, the drugs have been tested on healthy volunteers and have already had one go with people with Parkinson's so it is very unlikely that your partner or parent will be damaged in any way and it is most likely that it will help people come to some conclusion about the potential utility of the drug being trialled.*

Unfortunately, in our case, my husband had to stop the trial as we were not sure if it was affecting the drugs he was taking already and was actually making him worse. I think he got to the stage where his drugs were not working as well anyway. It has certainly not put him off as he is looking for another trial to sign up to.

It's a great way to learn about how your condition works, to understand the ways in which scientists are trying to treat it."

Joanna Goodburn

Why we made this guide

- In 2024, a group of people with experience of Parkinson's within Partners in Research worked together online to think about how we can better support people with Parkinson's in clinical trials research.
- Our Partners in Research felt that some of the stresses associated with clinical trials research could be reduced by having more information at the start.
- After an initial draft of this guide, we joined with other people with experience of Parkinson's to refine our work and create this document.
- We have created an online and paper-based resource to support people through clinical trials.
- There are a number of clinical trials available to people with Parkinson's , however, it can be very difficult to get onto a trial and trials can also end suddenly.
- There are two fundamental reasons why someone who wishes to be part of a clinical trial is unable to do so, or research may end suddenly: **Safety** and **Data Integrity**

"I was shocked when I was told I had Parkinson's. I am trying to be positive. Hopefully in time there will be more information and help for Parkinson's. I hope this helps anyone diagnosed with Parkinson's." ***Chris Culbertson***

Safety and Data Integrity

Safety

It is not ethical to include someone in a clinical trial if they are unlikely to benefit from the new treatment being studied, as this exposes them to risk of harm without the intended benefits i.e. it would compromise their safety.

Everything in a clinical trial is carefully planned and reviewed to protect your health and well-being at every stage.

Data Integrity

It is important that people participating in the trial are representative of people from whom the studied research is intended i.e. clinical trials on Parkinson's should include people with Parkinson's as participants.

The information that comes out of a clinical trial must be accurate, trustworthy and be relied upon for making decisions.

NHS SCOTLAND
NHS RESEARCH SCOTLAND
NEUROPROGRESSIVE AND DEMENTIA

Safety and Clinical Trials

- At the start of a clinical trial, researchers provide the study team with a list of characteristics that they are looking for known as the **inclusion or exclusion criteria** e.g. people over 60 years old, people with a study partner, people with capacity to consent etc.
- These criteria are very strict, and will be the same for each potential participant across the research. **The primary concern is whether the research will be safe.**
- It is also important that being involved in the research does not stop someone from continuing a treatment that is working for them i.e. if someone is taking a medication that is helping with nightmares, they would not be eligible for research into an alternative medication that has not yet been proven to be more effective than the one they are on.
- If the research has known side effects that may impact some people more than others, the research team would exclude people that would be more impacted by this i.e. if a medication has a side effect of lowering blood pressure, people who already experience low blood pressure would not be able to take part.
- The **pre-screening** and **screening** stages of clinical trials help the research staff to see whether a person meets the criteria.

Why are the criteria so strict?

- Figures suggest as much as 90% of drug development is unsuccessful.
- One of the reasons clinical trials have not found significant results in the past is because of too many differences between people in the group, which makes it harder to see whether something is having an effect.
- Therefore, strict inclusion or exclusion criteria are needed to try and 'uncloud' the picture and make the groups of participants as similar as possible.
- For example, if research thought that a physiotherapy intervention could improve mobility for people with Parkinson's but the test group was made up of people with better mobility scores than the control group to begin with, higher mobility scores after the intervention would be misleading.
- Similarly, if a new medication was found to helpful for people with specific **biomarkers**, you would need to know whether people had these biomarkers before they joined the research.
- Unfortunately, it is not always obvious whether someone will be able to take part. For example, some clinical trials include repeated brain scans to look at whether there are changes in the brain over time. If a potential participant experiences claustrophobia, they may find the scan process distressing, in which case they would not be asked to take part in the research.
- Before clinical research reaches potential participants, a series of research studies are performed with animal models (pre-clinical) and small pilot groups (early phase) to establish **safety and data integrity**.

"Have patience with what is going on as these things take time. People in charge of trial have put a lot of effort into it. May think 'what's all that about' but there will be a reason for it. Some of the questionnaires are long but go for them as every little helps. Still worth doing research as hope that the person asking the questions will get the answers for future generations. I enjoy the experience of clinical trials – some bits were tedious. It's of benefit to others in the future." - ***Alan Miller***

I have been involved in clinical trials. It's really important for me to be involved and help further understanding of Parkinson's. If you are looking to help with research then please go for it. You can get involved as much or as little as want. Still things at the other end of the spectrum that you can do to help.

It has been a positive experience for me and everyone is really pleasant, informative and has been good after-care and benefits for person doing is help further research. It was nice during coronavirus times to see a doctor and nurses- ***David Rigg***

Timeline of Clinical Research

A clinical trial can take around 10-15 years to go from initial testing to full approval for a new treatment.

- **The first priority is always safety.**
- Research can then look at whether it seems to make a difference, as well as continue to monitor safety/side effects.
- If the intervention appears to have an effect, research can then study whether this effect is better than what currently exists.
- If all of these stages go well, researchers then need to get approvals for the intervention to be used. It may be that an intervention is effective but only for a minority of people, or it may be very costly to deliver. Decisions then need to be made about whether it is the best use of resources.
- Clinical trials will each have different plans in place for if and when someone stays on a trial medication, and this should be explained clearly before deciding whether to take part.

Timeline of Clinical Research

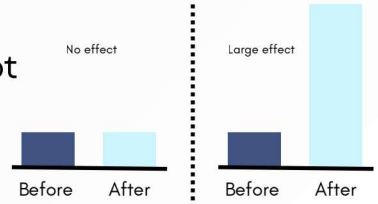
- Research can feel very busy when you're in it, but the follow on stage can feel very long and slow. People with Parkinson's want to have better communication from research teams about what is happening in the meantime.
- All to often research is written up for an academic audience but is not made accessible to all, in language or in availability.
- It also takes time to recruit suitable participants-
    - Research relies on people with Parkinson's being willing to put themselves forward for a trial despite the fact they may not be selected, it may not help them directly, or it may take a long time to conclude. All of which is a lot to ask of someone and their support network.
- Clinical research staff try and make the experience of being in research as positive as possible to balance out some of these challenges.

"I genuinely love working with people with Parkinson's in our clinical research studies. It's so important to me that their research journey feels positive, comfortable, and full of support–so they always know they're valued every step of the way."

Jessica Crossan- Senior Clinical Studies Officer NHS Lothian

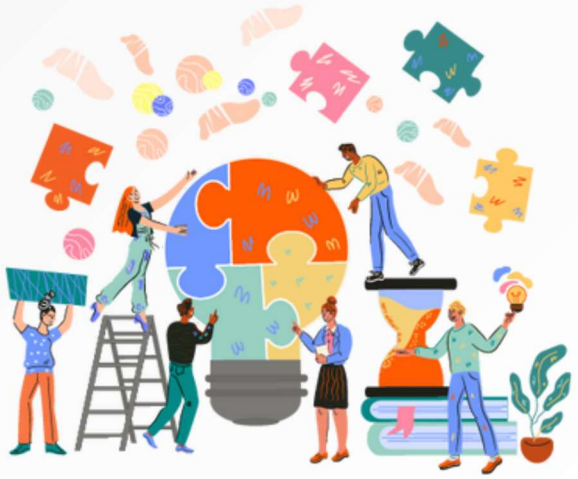
Finishing Clinical Trials

- There are lots of reasons why someone may leave a clinical trial early or a study may stop suddenly.
  - for example, a medication may cause side effects that had not previously been found e.g. dizziness which increases fall risk.
- It may be that the requirements of the research do not suit you e.g. too many appointments or difficulty taking the medication.
- If you have to leave a study or if finishes- **you have not failed!**- the research is just not right for you or others with Parkinson's.
- The priority is the safety and wellbeing of participants. Leaving a clinical trial will not change your clinical care.
- During the study someone may experience an **adverse event** or a **serious adverse event** which may mean it is not safe for others to stay in the intervention, even if they themselves have not experienced any difficulties. It depends on the seriousness of the event, the risk to the individual and how it impacts the study.
- If during the research trial, enough data shows that there is clearly no effect- it may be stopped early as it wouldn't be fair to keep people in a study for an intervention which is not going to work.
- A study ending early is not always bad news- it could be that the drug is shown to have such a clear positive effect that the placebo group are disadvantaged by not being able to take it!



Leaving a Clinical Trial

- Life happens! - We understand that life often unfolds in ways we may not expect
- Research should never be at the expense of clinical care or a person's wider quality of life
- Clinical trials can involve multiple visits across an extended period of time. We cannot always predict what else may come up for a participant in between.
- If your circumstances change, your research team will do what they can to support you to stay involved, but if the research stops being suitable for you it is important to remember-
  - **you have not let anyone down!**
- All research plans for a certain amount of 'drop-out'.
- Researchers include the potential for some participants not completing the trial when they decide how many people to recruit.
- By planning for some people leaving the study, it means that when someone needs to drop out, the research will still have enough people continuing to produce meaningful, impactful results.
- If your circumstances change and you are no longer able to be part of a clinical trial, it does not mean that you will not be able to participate in similar research in the future (if you would like to).



Feedback from research studies

- Clinical trials often require a lot of time, energy, and sharing of information about yourself with researchers.
- It can be frustrating that the information collected is not routinely shared with your primary healthcare team. In some cases, this may mean being asked to repeat tests that are done within a clinical trial outside of the research.
- Why is research data kept separate to health data?
  - A separation of routine care and research is important for reducing the potential for bias, as well as protecting the privacy and confidentiality of participants.
  - Clinical trials have strict rules for each step, and it is important that everyone is treated the same. Keeping information within the research team makes it easier to keep the process **standardised**, which is important for **data integrity**

- Feedback relating to the overall study is essential. Too often participants feel that they have been left with little to no information about a study that they have been a part of.
- It is important that researchers update participants throughout the study, including after the data collection has finished. It is okay to ask clinical trial research staff when you can expect updates.
- Clinical trials can take many years to complete, but regular updates and realistic timeframes can help participants to feel valued and included.
- A lack of feedback can also discourage people from taking part in another clinical trial in the future.

Acknowledgements

Thank you to all of the people with Parkinson's and people who support someone with Parkinson's who have joined us in co-creating this resource. Thank you to Joanna, David, Alan and Chris for sharing some of your experiences within the guide.

Thank you for NRS NDN staff including Denise Munro, Emma Fleming, Emma Law, Helen Davidson, Jacqui Kerr, and Jessica Crossan.

Thank you to Nikul Bakshi from Parkinson's UK for your involvement in the design and development of the guide.

Thank you to NHS Lothian Charity Fund for awarding funding for our in-person workshop as well as providing physical copies of the clinical trial guide for all contributors.

The Chief Scientist Office who fund the NRS Neuroprogressive and Dementia Network, who have supported dedicated funding for Partners in Research.

NHS SCOTLAND
NHS RESEARCH SCOTLAND
NEUROPROGRESSIVE AND DEMENTIA

Sign up to Partners in Research

Thank you for reading our clinical trial companion guide, co-created by people living with Parkinson's for people with Parkinson's.

Sign up to Research Interest Register

Thank you to NHS Research Scotland, The Chief Scientist Office, NHS Lothian Charity Fund and Parkinson's UK.

Contact: tay.ppipartners@nhs.scot