

## **Research Roundup**

This month, we share news that the phase 3 trial of repurposed drug, ambroxol, is underway and share the latest research news, events and opportunities to get involved.

## **Research news**

#### Phase 3 trial of ambroxol is underway

The first participant has received either ambroxol or the placebo in the ASPro-PD trial. This marks the start of 330 people with Parkinson's taking a component of an already approved drug over 2 years to see if it is beneficial.

In 2023 we announced we would be joining forces with Cure Parkinson's, alongside its strategic partners Van Andel Institute (VAI) and the John Black Charitable Foundation (JBCF) to co-fund a clinical trial called ASPro-PD looking at the potential of a cough medicine called ambroxol to treat Parkinson's.

After initial delays, we're now pleased to share that the first participant has started on the ASPro-PD study, which will involve them taking either ambroxol or a placebo (dummy) drug. This marks an exciting start for the study, which will assess whether ambroxol could slow or even stop the progression of Parkinson's.

#### What is ambroxol?

In 2009 researchers discovered that a medicine commonly used to treat coughs and sore throats, ambroxol, could also be used to treat Parkinson's.

Ambroxol boosts levels of an enzyme called GCase, which is known to help clear away waste products which have gathered in brain cells. In Parkinson's, a build up of a troublesome protein called alpha-synuclein is often seen in the brain tissue. It's thought that ambroxol may help improve the body's ability to clear away these clumps of alpha-synuclein and prevent damage to brain cells.

The ASPro-PD trial is a world-first phase 3 trial of ambroxol. Driven by Cure Parkinson's, following 8 years of work with the Parkinson's community, this £5.5m trial offers hope that a drug to slow the progression of Parkinson's may be on the horizon.

#### What are the next steps?

Over the coming months 330 people with Parkinson's will receive ambroxol or a placebo 'dummy' drug for 2 years. After this, all participants will be given ambroxol for 6 months. Neither participants nor their doctors will know whether they are receiving the trial drug or the placebo drug. Both ambroxol and the placebo drug will be taken in the form of a tablet 3 times a day.

As participants will be taking the drug for over 2 years, it's likely we won't know the results for a while. But this is a phase 3 trial, which is the last large study a drug must go through before it's decided whether it can be approved for use.

#### Professor David Dexter, Director of Research, said:

"It's very exciting to see the ASPro-PD trial move forward and start recruiting participants. This is the first large phase 3 trial that we have funded through the Parkinson's Virtual Biotech. If this is a success, ambroxol has the potential to be available in years and not decades."

#### How can I get involved?

The ASPro-PD research team is reaching out to people who are eligible to take part and who have already pre-registered through the PD Frontline study. This is a study where people are asked to supply a saliva swab, which is then studied to look for changes in certain genes.

People who meet the criteria for ASPro-PD, and have already taken part in PD Frontline will be invited to visit one of 15 study sites over England, Scotland and Wales. You will be contacted directly if you fit these criteria.

If you haven't already registered and taken part in PD Frontline, you may not be able to join the ASPro-PD study. However, you can still register to get genetically tested by PD Frontline and explore other research opportunities through the Take Part Hub: www.parkinsons.org.uk/take-part-hub

#### Ambroxol isn't currently recommended for Parkinson's

Whilst past trials have shown promise, there is still more research needed to show if ambroxol can be a safe and effective treatment for Parkinson's. This includes safe testing of dosages and frequencies. Parkinson's UK does not recommend you take any medications without a prescription. You can find answers to other questions about ambroxol and the ASPro-PD trial on the Parkinson's UK website:

#### www.bit.ly/FAQASPro

#### Progress towards stem cell treatment for Parkinson's

Research studies carried out in Japan and the US are exploring the potential of using stem cells to replace cells that are lost in the brain during Parkinson's.

#### What is a stem cell?

Every cell in the human body starts as a stem cell. Stem cells have the potential to become many different types of cell in the body. Depending on where it is, a stem cell will receive signals that give it the instructions to become the cell that's needed, for example a muscle cell, or a brain cell.

Stem cells are useful in Parkinson's research because they can be used to generate brain cells to study in the lab. Taking stem cells from the skin of people with Parkinson's and giving them the signals to become brain cells creates an ideal model for studying what might be happening in cells in the brain.

But researchers are also exploring whether stem cells could be used to replace brain cells that are lost in Parkinson's. Over time, brain cells of people with Parkinson's become damaged and start to die. These are important cells which produce a chemical called dopamine. As the cells die, levels of dopamine fall, and this leads to many of the symptoms associated with Parkinson's. Crucially, these cells are not naturally replaced. Although there are treatments that can

treat the symptoms of Parkinson's, there's currently nothing that can slow or stop the loss of these cells.

#### What does the latest research show?

The 2 studies that have shared their results used human stem cells that had been reprogrammed to become cells that produce the brain chemical dopamine. Participants in the study had surgery to inject the new cells into an area of the brain, and have been monitored closely.

The aims of both studies were to explore the safety of the stem cell surgery, and to check for any side effects.

The first study involved 7 people with Parkinson's in Japan, aged between 50 and 69. Everyone involved in the study was followed for 2 years. During this time, there was no report of any unwanted side effects, and scans showed that the injected cells were able to produce dopamine.

The second study followed 12 people with Parkinson's in the US. The researchers split the participants into 2 groups to test how many cells would be the optimal number to inject during the surgery without risking side effects. None of the participants experienced any severe side effects as a result of the treatment, regardless of whether they had received a low or a high dose of cells.

Some participants in both studies also reported that they had fewer movement symptoms after having the surgery. However, this varied among participants and would need further research and follow up to understand more clearly.

#### What happens next?

Claire Bale, Associate Director of Research, said: "Stem cell therapies hold huge promise for slowing, or even reversing, the progression of Parkinson's as they could be used to replace lost brain cells to potentially restore lost function.

"However, making stem cell therapies a reality has proven challenging. From a practical perspective, growing sufficient quantities of the right kind of cells to transplant, and from a safety perspective, making sure that transplanted cells don't cause side effects like dyskinesia, or worse, form tumours.

"It's really encouraging to see these findings from 2 separate early-stage trials. The results show that stem cell therapies can be delivered safely to people with Parkinson's, and also offer hope that the transplanted cells are starting to produce improvements in symptoms.

"There is still a lot more research to do but these studies are an important step forwards for stem cell therapies in Parkinson's."

# New funding for research exploring ways to manage Parkinson's symptoms

In our latest round of grants we've awarded funding for new projects that aim to improve symptoms of Parkinson's without the need for extra medication.

Alongside working to find better treatments, we know it's important that research also focuses on addressing the day-to-day challenges of living with Parkinson's. Our non-drug approaches grants provide funding for projects exploring new methods to improve symptoms of Parkinson's. We want to find new solutions to these challenges and improve everyday for people living with Parkinson's.

Diane is a research volunteer and part of a panel of people from the Parkinson's community who assess the applications, helping us choose which projects to fund.

**Diane shared:** "When looking at the non-drug applications you aren't looking at it only from your own personal needs, but the needs of all people with Parkinson's. You're looking for clarity in the application and the wider relevance

to all people with Parkinson's, and whether it will improve or influence day-to-day well being.

"So many projects have interested me, especially those on exercise. One of the projects I was particularly excited about this time was the one looking at speech and language. It opened my eyes to the lack of information and resources in this area for people with Parkinson's."

In the latest round of funding, we've awarded a total of £438,540 to 3 projects that are looking at addressing some of the highest priority symptoms for people with Parkinson's.

#### Our newly funded projects

### Treating language difficulties in Parkinson's

**Lead researcher:** Professor Anja Lowit, University of Strathclyde **Funding awarded:** £106,543

People with Parkinson's can often experience difficulties in communication. These include speech and voice problems, but also language difficulties such as finding the right word or understanding complex information. This study will explore how to best support people who experience language difficulties. Working with people with Parkinson's, loved ones and healthcare professionals, the team will develop a treatment programme that will be tested in a small study with people with Parkinson's and their support partners.

**Anja shared:** "We want to provide speech and language therapy beyond aspects of speech and voice, empowering people with Parkinson's to participate fully in daily activities and maintain greater independence."

#### Exploring the effects of ginger on gut health in Parkinson's

**Lead researcher:** Dr Caroline Williams-Gray, University of Cambridge **Funding awarded:** £199,467 Ginger is a household spice known to support gut health. It helps encourage healthy digestion, and reduces excessive inflammation, a process kickstarted in response to injury or infection but which can cause damage to the body if it's not controlled. Ginger also increases levels of a hormone called ghrelin which helps support gut health. Research has shown that people with or at a high risk of developing Parkinson's have lower levels of ghrelin. This study will explore whether ginger can improve ghrelin levels, inflammation, and gut symptoms for people with Parkinson's.

**Caroline shared:** "Gut symptoms are a major issue for many people with Parkinson's and may be linked to the condition getting worse over time. This new study will allow us to test whether ginger supplements can impact on both gut health and inflammation in Parkinson's."

#### Evaluating video-based training to help freezing of gait

**Lead researcher:** Dr William Young, University of Exeter **Funding award:** £132,530

Many people with Parkinson's experience difficulties walking, including freezing of gait. Freezing can mean that people feel 'glued' to the floor, which can be a very distressing symptom. Researchers at the University of Exeter worked with a group of people with Parkinson's and their loved ones to develop a series of videos that coached viewers on how to shift their weight and overcome freezing. The videos were tested in a small study, and 60% of participants found the videos helpful. Now, the team wants to further improve the videos and ensure the resources are accessible to a diverse audience across different communities.

**Will shared:** "We have thoroughly enjoyed working with our Project Advisory Group of people affected by Parkinson's and local branches in South West England to develop the ideas for this project. The aim is to provide accessible training that people can use in their own homes to successfully overcome 'freezing of gait' episodes and associated feelings of anxiety".

#### Par-Con 2025: save the date

We're delighted to share that we're bringing back Par-Con, our 2 day event about living well with Parkinson's. You'll be able to join us online or in person at The Vox, Birmingham on 24 and 25 October. Tickets will be available later in the summer.

## **Take Part in Research**

The development of new Parkinson's treatments is only possible if everyone is part of the research process. We need your help to push promising research forward.

#### Understanding how reduced facial expression affects communication

Researchers from the University of Suffolk are exploring how communication is affected by problems with facial expressions in Parkinson's. They want to understand how this affects well-being and the impact on people with Parkinson's and their loved ones.

#### Who do the researchers need?

- 15 people with Parkinson's who experience problems with facial expression. For example, a "masked" face or involuntary facial movements.
- 15 loved ones of people with Parkinson's who experience problems with facial expression. You must have known your loved one with Parkinson's before their diagnosis.
- Unfortunately, those with a history of stroke or Bell's Palsy are unable to take part.

#### What is involved?

• Taking part in an online or telephone interview with the researcher, which will last for up to 1 hour:

- The researcher will ask you questions about your experiences and the strategies you have found useful to help with communicating emotion
- The interview can be spread over multiple sessions if requested
- You will be reimbursed with a £50 voucher for taking part in the interview
- Following the interview, you will be invited to check a summary of the researcher's findings. If you check these, you will be reimbursed with a £25 voucher as a thank you for your time.

#### Interested in taking part?

To take part, please contact Abigail Webb by email at **a.webb6@uos.ac.uk** or phone **07545760585**.

The deadline for taking part in this research is **9 May 2025**.