

Research Roundup

This month we share news about progress in gene therapy research. Plus we give you a preview of what to expect at our upcoming community convention, Par-Con.

Research news

Early results show new gene therapy treatment is safe

11 people have received the treatment which aims to boost brain cells' ability to repair themselves.

Researchers in the US have shared their first results from a study looking at using a gene therapy, called AB-1005, to increase brain cells' production of a growth factor called GDNF. Gene therapy is a treatment that makes small changes to a person's genetic information within a specific type of cell. These changes enable cells to do more of, or start doing, a job. Such as, producing a growth factor.

What is a growth factor?

Growth factors are produced naturally by our cells to support the development, growth and survival of our bodies and our brains. They help to keep cells healthy and protect them from damage.

In Parkinson's, decades of research has focused on how growth factors could be used to help protect or even repair brain cells that have become damaged. This could help prevent the loss of brain cells over time, which could slow down Parkinson's getting worse.

GDNF, or glial derived neurotrophic factor, is one such growth factor that has been explored for its potential in Parkinson's. However, a problem with all growth factors is that they are very large, and can't easily get into the brain. This makes it very difficult to develop treatments that can reach the brain. For example, a simple pill containing GDNF wouldn't be effective. So, researchers are looking at other ways to deliver growth factors to where they need to be.

What did the researchers do?

The researchers at company AskBio have developed a type of treatment called gene therapy. The treatment is injected into the brain, and works by encouraging the nearby brain cells to start producing more GDNF. They do this by changing harmless viruses so that they can act as a delivery package to carry the key instructions required for producing GDNF. The virus can then enter targeted brain cells and insert the instructions to increase the production of the specific growth factor.

In this early study, 11 people with Parkinson's were given the AB-1005 gene therapy treatment by injection. They were then monitored for 18 months.

What did the results show?

The results published showed that there were no serious side effects for any of the 11 people who had undergone the treatment, after 18 months.

Some people in the study (5 out of 11) also saw an improvement over the 18 months in their movement symptoms of Parkinson's. For the other participants, movement symptoms remained stable over this time.

The full results of the small study are available on the International Parkinson and Movement Disorders Society website.

What happens next?

The research team is now working on the next stage of the study. This will look to recruit over 80 people with Parkinson's worldwide, including in the UK. It will be a double blinded study, with half the participants having the AB-1005 therapy, and the other half having the same injection but not receiving the treatment. This is the gold standard for testing a new treatment. It ensures that the results can't be impacted by the participants or researchers knowing whether or not they have been given the drug.

Dr Becky Jones, Research Communications Manager, said:

"It's exciting to see positive early results of this gene therapy study. This is one of many research studies exploring gene therapies and growth factors for Parkinson's, which makes it an exciting field for Parkinson's research. We look forward to seeing the results of this next, larger study, where we'll have more of an idea of how well the therapy might be working to slow the progression of Parkinson's."

Dr Lynsey Bilsland joins our Virtual Biotech team

We're excited to share that Dr Lynsey Bilsland has been appointed Managing Director of our Parkinson's Virtual Biotech. Lynsey brings a wealth of knowledge and experience, and shares our passion for delivering new treatments in years, not decades.

Lynsey said: "Being agile and patient-led keeps the Virtual Biotech focused on investing in the most promising treatments for the biggest unmet needs for people with Parkinson's, and that's what drives me in my work. I can't wait to get started at the end of July."

Professor Miratul Muqit to lead new Parkinson's Research Centre

We joined forces with the UK Dementia Research Institute (UK DRI) to launch a new £10m Parkinson's research centre. We're delighted to share that Professor Miratul Muqit, a world leader in Parkinson's research, has been appointed to lead the centre.

Miratul said: "It is an absolute honour to lead the new UK DRI Parkinson's Research Centre in partnership with Parkinson's UK. In my role I will make the centre a beacon for open science and collaboration with researchers all over the UK and globally to deliver for people with Parkinson's."

1 July 2025

Par-Con registration opens next month!

We have been busy organising Par-Con 2025, our 2-day community convention for people affected by Parkinson's. The event, which has been planned and designed alongside a group of people with Parkinson's and their loved ones, will bring people together to connect and learn.

From a live podcast recording, to creative taster sessions, to a fantastic line up of speakers covering the latest research, there will be something for everyone.

Par-Con will take place on **24 and 25 October** in **The Vox in Birmingham**. You can join in-person or online, and all sessions will be recorded.

We will be sharing details of how to register on Friday 25 July.