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Gene therapy for Parkinson's

The treatment is delivered directly to the brain

Doctors are to begin the first ever trial using gene therapy to treat Parkinson's Disease.

They hope gene therapy will be more effective and less invasive than the medical or surgical treatments currently available, which can be linked to side effects.

And they say it may even be possible to halt the progress of the incurable degenerative condition.

Around 120,000 people in the UK have Parkinson's.

The techique uses gene therapy to "re-set" a specific group of cells in the brain which have become overactive.

> **It may arrest or delay disease progression**
>
> **Dr Matthew During, University of Auckland**

This causes the impaired movement and other symptoms associated with Parkinson's.

The team of scientists, from New Zealand and the US have carried out successful tests in rats, and hope to begin the first trials in humans by the end of the year.

Twelve patients who have had severe Parkinson's for at least five years, and for whom current therapies are no longer effective, will take part.

Movement control

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of a specific group of nerve cells deep in the brain which make the signalling molecule dopamine, which affects how the brain controls movement.

A region at the centre of this network, called the subthalamic nucleus (STN), becomes extremely overactive in patients with Parkinson's.

If this can be silenced, patients see a dramatic reduction in their symptoms.

"It is very encouraging for people with Parkinson's"

Robert Meadowcroft, Parkinson's Disease Society

The gene therapy uses the GAD gene, which makes a small molecule called GABA. This is released by nerve cells to inhibit, or dampen activity.

Scientists deliver this directly to the overactive cells by inserting the GAD gene into a modified virus.

This gene therapy "re-sets" the overactive cells, and brain activity becomes more normal.

Tests on rats showed the GAD gene was present and producing GABA as anticipated.

Behaviour tests showed they had retained more normal function and did not develop further signs of Parkinson's compared to rats who had not been given the gene therapy.

In addition to work in rats, tests on monkeys have shown the therapy was safe and non-toxic.

Chemical signals

Dr Matthew During, professor of molecular medicine at the University of Auckland, who is leading the research, said: "We are very encouraged that in addition to the effect this therapy has on quieting symptoms, we present evidence that suggests it may arrest or delay disease progression.

Follow researcher Dr Michael Kaplitt, a specialist in neurosurgery at Weill Cornell Medical College, New York, said: "Both surgical and medical treatments have certain side effects.

"We use gene therapy to adjust the chemical signalling of these brain areas to a more

normal setting.

"This exploits the best parts of current therapy but makes it more powerful, less invasive and potentially safer."

Robert Meadowcroft, director of policy, research and information at the UK's Parkinson's Disease Society, told BBC News Online: "I think it is very encouraging for people with Parkinson's that this trial is going forward.

"In terms of conventional treatment for Parkinson's, most people will have drug treatment.

"In the first stages, that can be very effective. But after four or five years the side effects, such as dyskinesias, or unwanted movements, can become quite distressing themselves."

He said surgery would only be suitable for a small proportion of Parkinson's patients.

Mr Meadowcroft added: "This research sets out some very promising findings from animal studies, and therefore we look forward with hope, but caution."

The research is published in the magazine Science.

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