

Improved stem cell approach could aid fight against Parkinson's

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Scientists have taken a key step towards improving an emerging class of treatments for Parkinson's disease.

The advance could markedly improve a next generation of therapies for the condition, which affects around one in 350 people in the UK.

It could aid development of the promising treatment—known as cell replacement therapy—which was first used in a clinical trial this year. Experts hope the approach, which involves transplanting healthy [cells](#) into parts of the brain damaged by Parkinson's, could alleviate symptoms such as tremor and balance problems.

The latest development addresses limitations in the treatment in which, over time, transplanted tissue can acquire signs of disease from nearby cells.

Researchers at the University of Edinburgh have created [stem cells](#)—which have the ability to transform into any cell type—that are resistant to developing Parkinson's.

They snipped out sections of DNA from [human cells](#) in the lab using advanced technology known as CRISPR. In doing so, they removed a gene linked to the formation of toxic clumps, known as Lewy bodies, which are typical of Parkinson's brain cells.

In lab tests, the stem cells were transformed into brain cells that produce

dopamine—a key brain chemical that is lost in Parkinson's—in a dish. The cells were then treated with a chemical agent to induce Lewy bodies.

Cells that had been gene-edited did not form the toxic clumps, compared with unedited cells, which developed signs of Parkinson's.

Researchers say the advance could be most beneficial to younger patients living with Parkinson's and those with an aggressive form of the condition, but that the advance had to be tested in human trials.

The study, published in the *European Journal of Neuroscience*, was funded by the UK Centre for Mammalian Synthetic Biology, the pharmaceutical company UCB Biopharma and The Cure Parkinson's Trust.

Dr. Tilo Kunath of the Medical Research Council's Centre for Regenerative Medicine at the University of Edinburgh, who led the study, said: "We know that Parkinson's disease spreads from neuron-neuron, invading [healthy cells](#). This could essentially put a [shelf life](#) on the potential of cell replacement therapy. Our exciting discovery has the potential to considerably improve these emerging treatments."

Dr. Simon Stott, Deputy Director of Research for the Cure Parkinson's Trust said, "Cell replacement therapy represents one experimental approach to [regenerative medicine](#) for people with Parkinson's. This new research by Dr. Tilo Kunath and his team at the University of Edinburgh provides another advancement in the development of this treatment. The Cure Parkinson's Trust is thrilled to be associated with this inspiring and [innovative research](#)"

More information: Yixi Chen et al, Engineering synucleinopathy-resistant human dopaminergic neurons by CRISPR-mediated deletion of the SNCA gene, *European Journal of Neuroscience* (2018). [DOI:](#)

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