

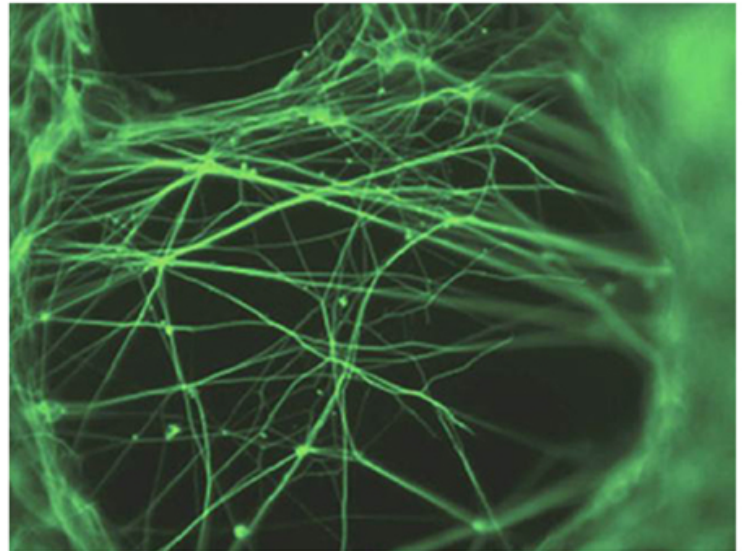
Parkinson's disease: how could stem cells help?

What do we know?

Tremors, muscle rigidity and other symptoms of Parkinson's disease (PD) are caused by the death of dopamine-producing neurons in the brain. Dopamine-producing neurons throughout the brain are affected, but the 'substantia nigra' is the primary brain region where neurons are lost.

People affected by PD often develop abnormal protein clumps in their brain called Lewy bodies. These clumps are made of a protein called alpha-synuclein.

Levodopa (L-DOPA) is the primary drug used to treat PD. Levodopa is converted into dopamine when in the body, which compensates for lost dopamine-producing neurons.



Nerve cells grown in the lab. A green dye was used to label any type of neuron in the sample.

Photo: Tilo Kunath, University of Edinburgh.

What are researchers investigating?

Approximately 5% of people with PD have inheritable gene mutations linked to PD. Researchers are investigating what causes PD in the other 95% of patients in clinical studies, animal models and cell models.

Transplantation of young brain cells from human foetuses into people with PD has shown promising results in previous clinical trials. The current TRANSEURO study is re-examining this treatment method with the aim of minimising side effects and measuring efficacy.

Scientists can now make dopamine-producing neurons from both human embryonic stem cells (ESCs) and human induced pluripotent stem cells (iPSCs). Neurons made from human ESCs and iPSCs mature into human dopamine-producing neurons, survive and function after transplantation into mouse, rat and monkey models of PD.

What are the challenges?

Alpha-synuclein and many other proteins coded by genes linked to PD are still poorly understood. These genetic forms of PD still only make up a small proportion of patients. This makes it very difficult to understand the precise causes of PD.

Although the medications we have for PD are very useful, they begin to lose effectiveness after several years and as the disease progresses. Stem cell treatments potentially offer a way to provide new neurons that can replace the neurons lost to the disease. Stem cell treatments based in human ESCs and iPSCs are still not approved for the treatment of PD in humans, however the first clinical trials are expected to start in 2018. Studies in animal models of PD have demonstrated that human dopamine neurons from ESCs and iPSCs are safe, effective and similar enough to the original human nigral neurons. However, these still need to be shown that they are safe and beneficial to people with PD before they will be widely used.