

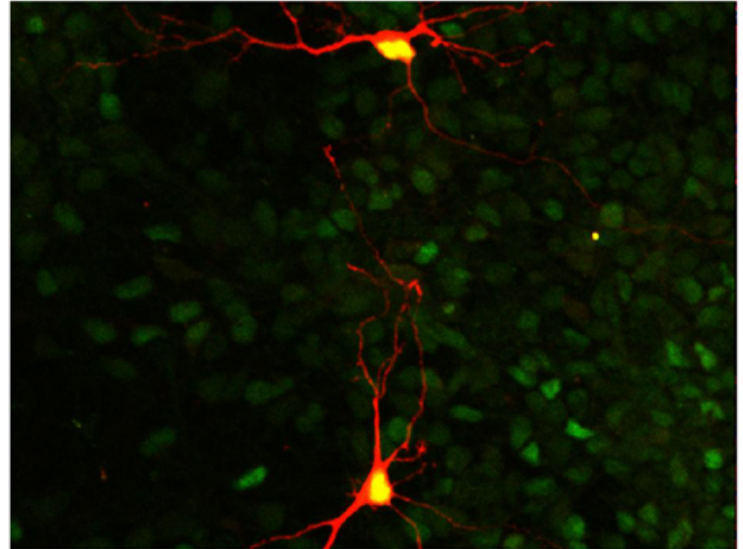
# Huntington's disease: how could stem cells help?

## What do we know?

Huntington's disease (HD) is a genetically heritable disease that destroys 'medium spiny neurons' (MSNs) in the brain, leading to emotional changes, mental decline and impaired movement. Symptoms often appear in patients between the ages of 35-50, but can start earlier.

HD is caused by a 'CAG' sequence of DNA being repeated >40 times in the Huntingtin gene, with people with >35 repeats at risk of developing HD. There are currently no treatments for Huntington's disease.

Stem cells have been an invaluable tool for studying many aspects of HD, from how HD causes MSNs to die to testing new treatments.



Human medium spiny neurons grown in the lab from embryonic stem cells.

Photo: Charles Arber, University College London

## What are researchers investigating?

The Huntingtin gene makes a protein important for neurons, but scientists are still trying to understand what this protein does in cells and why too many 'CAG' repeats causes MSNs to die.

Researchers are using MSNs made with induced pluripotent stem cells (iPSCs) to model HD and observe how HD progresses. iPSCs are also used to rapidly test new HD drugs, gene therapies and other treatments faster than before iPSCs were available.

Studies are examining how brain (neural) stem cells could be transplanted in patients' brains to repair the brain and possibly regrow MSNs and other needed brain cells.

## What are the challenges?

Currently there are no approved stem cell treatments for HD. Neural stem cell transplants have undergone several trial studies for treating HD patients, but these treatments must still undergo rigorous clinical trials to show they are safe and effective.

Ethical and reliable sources of neural stem cells must be developed. iPSCs and embryonic stem cells may be able to make large numbers of neural stem cells, but these cells must be uniform, predictable and safe. If they are not properly tested pluripotent stem cells could cause cancer or other life-threatening complications.