PARKINSON'S^{UK} CHANGE ATTITUDES. FIND A CURE. JOIN US.

Parkinson's UK policy statement Stem cell research

"I am interested in stem cell research but understand that implanting dopamine stem cells has not proved successful. Yet I hope that this area of research will continue as it appears to be a very important possibility for the future."

Person with Parkinson's

What we believe

Stem cell research has the potential to lead to new and better treatments for Parkinson's. We understand the sensitive issues around certain areas of stem cell research, and respect the views of those concerned by them, but we firmly support the continuation of stem cell based research within the rigorous ethical and regulatory framework in place in the UK. Continued, well-regulated research could benefit many thousands of people affected by conditions such as Parkinson's.

Why we believe this

Promising area of research

Stem cells have the potential to develop into any kind of cell found in the body, including brain cells.¹ This means that stem cells could be used to treat a wide range of conditions, including Parkinson's, where new cells could be used to repair and replace damaged tissue.

Scientists are able to turn stem cells into dopamine-producing nerve cells – the type of brain cells affected in Parkinson's.² As we are able to generate and study these specialised nerve cells, research of this kind allows us to develop our understanding of Parkinson's and how to treat it.

Being able to transform stem cells into dopamine-producing cells also gives us hope that future treatments may be able to replace damaged cells in the brain. Trials aimed at investigating the ability of transplanted nerve cells to reverse Parkinson's are ongoing.³

Where do researchers get stem cells from?

Existing sources of stem cells

Currently, researchers can get stem cells from several sources, including embryos, blood cells taken from the umbilical cord at birth and bone marrow.⁴ Stem cells can also be found in adult tissue, and recently researchers have developed a way to convert adult cells, such as skin cells, into stem cells.⁵

1 Lunn SJ et al (2011) 'Stem cell technology for neurodegenerative diseases' Annals of neurology 70.3:353–361

2 Bjorklund A and Jeffrey KH (2013) 'Cell therapy for Parkinson's disease: what next?' Movement Disorders 28.1:110-115

3 Yasuhara T et al (2015) 'Regenerative medicine for Parkinson's disease' Neurologia medico-chirurgica 55.2:113

Embryonic stem cells

In the UK, stem cells from human embryos can be collected from embryos produced as part of in vitro fertilisation (IVF) programmes that would otherwise be destroyed. There are strict legal guidelines for using embryonic cells for research from the Human Fertilisation and Embryology Authority (HFEA).

These guidelines are based on UK government legislation. The Human Fertilisation and Embryology Act 1990 states that only very early-stage embryos can be used in research – they cannot be more than 14 days old.⁶

Embryonic stem cells show great promise to help develop a treatment for Parkinson's but much more research is needed in order for scientists to understand how stem cells work. By understanding how these cells develop into the different types of cells in the human body, we may be able to develop treatments for Parkinson's and other medical conditions.

Adult stem cells

Adult stem cells are found in small numbers in certain parts of the body – they are activated when tissue is damaged and make new cells to repair the tissue. Adult stem cells are less flexible than embryonic stem cells as they cannot turn into any cell in the body.⁷ For instance, liver stem cells can turn into a number of different cells found in the liver but they cannot turn into brain cells.

While research using adult stem cells has produced promising results for a number of conditions, they present many challenges in Parkinson's research. Adult stem cells that can turn into brain cells are only available in a few places in the body.⁸ This makes it difficult to collect the number of cells needed for research or therapy.

Alternative way of making stem cells iPS cells

At the end of 2007, researchers discovered a way of turning adult cells, such as skin cells, back into 'blank' stem cells.^{5,9} They called these reprogrammed cells iPS cells and found that they behave in a very similar way to embryonic stem cells.¹⁰ In 2012, the scientific community recognised this discovery by awarding two scientists involved The Nobel Prize in Physiology or Medicine.

iPS cells have the potential to turn into any type of cell in the adult body. And scientists are able to turn these cells into the type of nerve cell lost in Parkinson's. While we need to develop how we turn them into dopamine-producing nerve cells, transplanting these cells into the brain could potentially reduce the symptoms of Parkinson's.¹¹ Animal models where these cells have been transplanted have shown decreased symptoms.

However, iPS cells are not identical to embryonic stem cells and we still need to know more about these differences. Scientists don't know if there are any long-term harmful effects that could be caused by the reprogramming of cells into iPS cells. We also still don't know if cells made from iPS cells are stable over long periods of time, as they may change into other types of cell.^{12,13} However, more recent research has found new ways to make iPS cells that scientists hope will be safer.¹⁴

There are currently no specific legal restrictions on the use of adult stem cells or iPS cells although general legal restrictions on the use of human tissue may apply.¹⁵

Backed by the majority

Public support for this type of research is strong. The 2014 Public Attitudes to Science survey showed that 57% of UK citizens feel the benefits of stem cell research outweigh any potential risks.¹⁹

A further public consultation exercise conducted by the HFEA in 2007 showed that 79% of people support the use of human embryos for medical research to find treatments for conditions such as Parkinson's.²⁰ We are also aware of a high level of support for stem cell research from Parkinson's UK members.²¹

- 4 Gögel S et al (2011) 'Progress and prospects: stem cells and neurological diseases' *Gene Therapy* 18:1–6
- 5 Takahashi K et al (2007) 'Induction of pluripotent stem cells from adult human fibroblasts by defined factors.' *Cell* 131:861–872 6 Human Fertilisation and Embryology Act 1990 (as amended) Activities covered by the Act. Section 3
- www.legislation.gov.uk/ukpga/1990/37/section/3 (accessed: 18 May 2016)

8 Fuentealba LC et al (2012) 'Adult neural stem cells bridge their niche' Cell Stem Cell 10.6:698-708

⁷ Lodi D et al (2011) 'Stem cells in clinical practice: applications and warnings' J Exp Clin Cancer Res 30.9:1-20

What Parkinson's UK is doing

Progress towards new and better treatments

Continuous progress is being made towards cell replacement therapies for Parkinson's.^{16,17} Parkinson's UK is committed to making more new and better treatments a reality, and is funding the iPS brain cell bank at the Oxford Parkinson's Disease Centre.

In work funded by Parkinson's UK, iPS cells have been created from people with Parkinson's.¹⁸ These cells can be used to better understand how changes in DNA can affect dopamine producing nerve cells in Parkinson's. And nerve cells made from iPS cells can also be used to test drugs and treatments.

We continue to campaign to ensure all avenues of research remain open within the UK's strict regulatory framework.

Acknowledgement

We are grateful for the advice and guidance of our Policy Panel and Research Support Network in shaping this position paper. The Policy Panel consists of people with experience of Parkinson's who meet on a regular basis to help guide the charity's position on a range of policy issues. The Research Support Network is a group of around 2,000 people with an interest in Parkinson's research.

Further information

Please contact the Policy and Service Improvement team on 020 7963 9394 or email campaigns@parkinsons.org.uk

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11 Rhee YH et al (2011) 'Protein-based human iPS cells efficiently generate functional dopamine neurons and can treat a rat model of Parkinson disease' *J Clin Invest* 121:2326–2335

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14 Stadtfeld M et al (2008) 'Induced pluripotent stem cells generated without viral integration' Science 322(5903):945-949

15 Human Fertilisation and Embryology Act 1990 (as amended) <u>http://www.legislation.gov.uk/ukpga/1990/37</u> (accessed: 18 May 2016) 16 Arenas E (2010) 'Towards stem cell replacement therapies for Parkinson's disease' *Biochemical and biophysical research communications* 396.1:152–156

17 Smith Y et al (2012) 'Parkinson's disease therapeutics: new developments and challenges since the introduction of levodopa' *Neuropsychopharmacology* 37.1:213–246

18 Devine MJ et al (2011) 'Parkinson's disease induced pluripotent stem cells with triplication of the α-synuclein locus' *Nat Commun.* 2:440 19 Ipsos MORI 'Public Attitudes to Science 2014' https://www.ipsos-mori.com/researchpublications/researcharchive/3357/Public-Attitudes-to-Science-2014.aspx (accessed: 18 May 2016)

20 Human Fertilisation and Embryology Authority 'Hybrids and Chimeras: A report on the findings of the consultation' (2007) http://www.hfea.gov.uk/519.html

21 Parkinson's UK Life with Parkinson's today - room for improvement (2008) http://www.parkinsons.org.uk/content/life-parkinsons-today-room-improvement