

# Parkinson's UK policy statements: 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 bring forward 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.<sup>1</sup> 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 now able to turn stem cells into dopamine-producing nerve cells – the type of brain cell affected in Parkinson's.<sup>2,3</sup> 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 to improve the motor symptoms of Parkinson's. Indeed, early stage clinical trials to test the safety and effectiveness of stem cell therapies in people with Parkinson's are already underway.<sup>4</sup>

In Parkinson's, pioneering clinical trials to understand the potential for cell transplantation have required the use of human fetal brain tissue.<sup>5-7</sup> With advances in stem cell research, the ability to generate new brain cells from stem cells also has a role in making cell

transplant therapies more widely available while addressing some of the ethical concerns associated with this area of research.<sup>8</sup>

While progress is being made, it's important to note that no stem cell based treatment has yet been proven to be safe and effective for the condition. Some private clinics offer stem cell therapies, but the safety and effects of these treatments are unknown. We strongly encourage people to speak to their Parkinson's specialist or the Parkinson's UK research team if they are considering this kind of therapy.

## Where do researchers get stem cells from?

### Existing sources of stem cells

Stem cells can be obtained from several sources, including embryos, blood cells taken from the umbilical cord at birth and adult bone marrow.<sup>9</sup> With developments in stem cell research, it is now also possible to convert adult cells, such as skin cells, into stem cells.<sup>10,11</sup>

### 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.<sup>12</sup>

As these cells have a natural ability to develop into other types of cells in the body, their potential for treating neurodegenerative conditions, like Parkinson's, has been investigated.<sup>4</sup> Studies in animal models have shown that it may be possible to use embryonic stem cells to generate the type of nerve cell lost in Parkinson's and transplant them into the brain to reverse the symptoms of the condition. However, more research is needed to understand the safety, long term risks and potential benefits and various trials are underway in people with Parkinson's.<sup>4</sup>

### 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.<sup>13</sup> For instance, stem cells from the bone marrow can be turned into different types of blood cells but may have limited potential when turned into other types of adult cells like brain cells.<sup>14</sup>

While research using adult stem cells has produced promising results for a number of conditions,<sup>15,16</sup> fewer studies have investigated their potential for Parkinson's. With potential limitations using adult stem cells to make dopamine producing nerve cells,<sup>14</sup> clinical trials are underway looking at the benefits when these stem cells are infused into the bloodstream.<sup>17</sup>

## 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.<sup>10,11</sup> They called these reprogrammed cells iPS cells and found that they behave in a very similar way to embryonic stem cells.<sup>18</sup> 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. Transplanting new dopamine-producing cells made from iPS cells into the brain could potentially reduce the symptoms of Parkinson's. Indeed, animal models where these cells have been transplanted have shown decreased symptoms and have led to first in man trials.<sup>4</sup>

However, scientists are still concerned about possible long-term harmful effects that could be caused by reprogramming cells. While newer techniques seem to improve the stability of cells over time,<sup>19</sup> and reduce the risk of tumours forming,<sup>20,21</sup> there is a continued need for caution as therapies based on reprogramming cells move towards clinic.

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.<sup>22</sup> There may be a need for a review of the ethics and consent for stem cell research for both data and tissue and sharing options through research in uk and across the world.

## What Parkinson's UK is doing

### Progress towards new and better treatments

Continuous progress is being made towards cell replacement therapies for Parkinson's.<sup>4,25,26</sup> Parkinson's UK is committed to making more new and better treatments a reality. We've invested millions in cutting-edge stem cell research, which has advanced our understanding of how stem cells could be used to treat Parkinson's directly, and also as a tool for testing and developing other new treatments.

- In work funded by Parkinson's UK, iPS cells have been created from people with Parkinson's.<sup>27,28</sup> These cells can be used to better understand how changes in DNA can affect dopamine producing nerve cells in Parkinson's and can also be used to test drugs and treatments.

- In the early 1990s, we helped fund early cell transplantation trials in a small group of people with Parkinson's. This research has paved the way for stem cell therapy trials underway today.
- Research being funded by the charity in 2021 aims to better understand the potential for stem cell transplant therapies to improve the non motor symptoms of Parkinson's. Recognition of the need to increase research into non motor symptoms is a priority. These symptoms have major repercussions on the quality of life and mental health of people with Parkinson's.

We will continue to call on the Government for greater investment to ensure the research community has the funding it needs to make breakthroughs in Parkinson's treatment and ultimately find a cure, through a well-regulated research framework which considers stem cell research as an option to support the discovery of more effective treatments.

Members of the community have also told us they feel there is a need to increase research into non- motor symptoms as a priority as they have major repercussions on quality of life and mental health. To note, this statement stands part of a broader policy stance on research which can be found in other policy statements.

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## Further information

Please contact the Policy and Campaigns team on 0207 932 1323 or email [campaigns@parkinsons.org.uk](mailto:campaigns@parkinsons.org.uk).

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