Gene therapy for Parkinson's

This information sheet sets out what gene therapy is and how it is important in finding a cure for Parkinson’s. It also looks at current research into new types of gene therapy that may become treatments.

What is Parkinson’s and what causes it?

Parkinson’s is a progressive neurological condition. This means that it gets worse over time and it’s caused by a problem in the brain.

People with Parkinson's don’t have enough of a chemical called dopamine because some nerve cells in their brain that make it have died. Because of the loss of these dopamine producing cells, the symptoms of Parkinson’s appear. These include tremor, rigidity and slowness of movement.

We don’t yet know why people get Parkinson’s, but researchers suspect it’s a combination of genetic and environmental factors that cause dopamine producing nerve cells to die.

Current treatments for Parkinson’s try to replace lost dopamine by using drugs that work like dopamine. Gene therapy research is trying to find a way to prevent the remaining nerve cells from dying, restore nerve cells or replace them.

What are genes?

A gene is a short segment of DNA that provides our cells with the instructions for making protein. Humans have around 23,000 genes, which contain the blueprint for creating all the proteins required to produce the cells, tissues and organs that make up our bodies.
We all have our own unique mixture of genes that we inherit from our parents. And this is what makes us all different. These natural variations affect everything from our personality to our height, as well as how likely we are to develop certain illnesses or conditions.

**What is gene therapy?**
Gene therapy is a new approach to treating medical conditions that uses genes instead of drugs. The most common form of gene therapy involves inserting a working gene into the cells of a patient in order to treat or to try to prevent the cells from dying. These genes would work by instructing the cells to produce things, such as proteins, to help treat your condition or its symptoms.

So gene therapies use genes to change the way certain cells behave – to stop them doing something that could be harmful or to make them do something that is beneficial.

With gene therapy it is hoped that Parkinson’s will eventually be halted or even reversed and a possible cure or cures found.

**Why are gene therapies being developed?**
During the early stages of Parkinson’s, drugs such as levodopa and dopamine agonists can be very effective in treating the symptoms. But they are not a cure and cannot slow down the progression of the condition. With long-term use, the drugs start to become less effective and you can get more side effects. The symptoms of Parkinson’s also tend to become worse as the condition progresses.

Gene therapies have the potential to change the way nerve cells work. So these therapies could be used to slow down or even stop the progression of Parkinson’s. For example, they could be used to get cells to produce more dopamine and to stop them from dying.

**What kinds of gene therapy are being developed?**
Using gene therapy, scientists aim to put various kinds of genes directly into the affected nerve cells in order to treat Parkinson’s. There are three main types of gene therapies that may be used:

- **Growth factors**
  These factors promote growth and survival and can help to protect nerve cells from further damage. Putting genes for growth factors into the brain has been attempted in trials with people with Parkinson’s. But some major side effects were reported. More research is needed to improve this technique before it could be developed as a treatment.

- **Proteins that increase the levels of dopamine production**
  Gene therapy could be used to instruct cells in the brain to produce their own dopamine. The result should be that dopamine levels are brought back towards a normal level within the part of the brain that has been treated and so help to reduce symptoms.

- **Restoring the activity of nerve cells in the brain**
  In Parkinson’s the nerve cells in the basal ganglia area of the brain can become overactive. This region helps to control movement so any change in this area will have an effect on your movements. This overactivity can be lessened by a chemical produced in the brain called GABA. But this chemical is lacking in the brains of some people with Parkinson’s.

  In this type of therapy, a gene can be inserted to instruct the cells in the basal ganglia to make GABA. This in turn may reduce the over-activity of the nerve cells in this part of the brain.

**How can genes be delivered into the brain?**
In order to transfer genes into the brains of people with Parkinson’s, researchers are using viruses. They can be used to carry genes to a particular place in the body or brain. But before the virus can be used as a carrier it needs to be changed so that it can’t carry infection, then it can carry any therapeutic materials needed around the body safely.

These viruses have been tested in several animal models of Parkinson’s and the trials have so far shown that it may be possible to prevent nerve cell death and to slow, stop or reverse Parkinson’s symptoms.
What further research needs to be done?
So far trials show that gene therapy seems to be safe but there are several things that need to be addressed before gene therapy can become a treatment for neurological conditions such as Parkinson’s in humans.

These include:
• making sure it is better than current treatments and side effects are kept to a minimum
• ensuring it will last in the brain for a long time, hopefully for many years
• making sure it will be safe and stay in those areas in the brain where it is needed
• knowing how much is needed to be effective and of the greatest benefit
• completely investigating risks and other side effects
• running studies to see which people with Parkinson’s will benefit from gene therapy

Is gene therapy available as a treatment?
Gene therapy is not yet available as a treatment option for people with Parkinson’s and is still in the early stages of clinical research. There are trials currently taking place in humans for new gene therapies for Parkinson’s. These trials are at different stages of development. While most results appear to be encouraging, more research needs to be carried out before we can say if this will be available as a treatment for people with Parkinson’s.

For now what we can say is that gene therapy has a real potential to make an impact on treatment for people with Parkinson’s.

Where can I get more information on gene therapy?
We produce the twice-yearly research magazine Progress, which looks at Parkinson’s UK-funded research in more depth. This is sent to all members.

To find out more about membership contact our membership department: membership@parkinsons.org.uk, parkinsons.org.uk/join 020 7932 1344.

Our Research Support Network brings together people driven to help find a cure and better treatments for Parkinson’s. Through our network, anyone can get involved in research and raise funds and awareness for Parkinson’s research. If you would like to know more about how you can get involved in supporting research, contact the Research and Innovation team at research@parkinsons.org.uk or call 020 7963 9326.

We list UK clinical trials on our website parkinsons.org.uk/researchstudies.

More information and support

Parkinson’s nurses
Parkinson’s nurses provide expert advice and support to people with Parkinson’s and those who care for them. They can also liaise with other health and social care professionals to make sure your needs are met.

Parkinson’s nurses may not be available in every area, but your GP or specialist can give you more details on local services.

Information and support from Parkinson’s UK
You can call our free confidential helpline for general support and information. Call 0808 800 0303 (calls are free from UK landlines and most mobile networks) or email hello@parkinsons.org.uk.

Our helpline can put you in touch with one of our local information and support workers, who provide one-to-one information and support to anyone affected by Parkinson’s. They can also provide links to local groups and services.
Our website has information about your local support team and how to contact them at parkinsons.org.uk/localtoyou. You can find details of our local groups and your nearest meeting at parkinsons.org.uk/localgroups. You can also visit parkinsons.org.uk/forum to speak with other people in a similar situation on our online discussion forum.

Glossary of terms

Proteins
Large molecules in the body that have a variety of functions, including making up the structure of many body tissues, enzymes that promote biochemical reactions in the body, and hormones.

Virus
One of the smallest known types of infectious agents. Viruses can also be used by scientists as carriers to transport therapeutic materials, such as adapted genes, around the body.

Basal ganglia
A part of the brain involved in co-ordinating movements.

GABA or gamma aminobutyric acid
A neurotransmitter that slows down the activity of nerve cells in the brain.

Dopamine
A chemical messenger produced by cells in specific parts of the brain. It transmits messages that play an important role in the co-ordination of movement.

Levodopa
A chemical that occurs naturally in the brain and is converted into dopamine (the chemical messenger lacking in Parkinson's).

Dopamine agonists
A group of drugs used to treat Parkinson's that mimic the effects of the chemical messenger dopamine which is depleted in Parkinson's.

Gene
A section of DNA containing the instructions for a particular thing (e.g. eye colour, making an enzyme).

Gene therapy
Manipulating or adding genes to cells to help them work more effectively, for instance produce more dopamine.

Genetics
The study of heredity and variation and the nature of genes.
Thank you to everyone who contributed to and reviewed this information sheet:

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Thanks also to our information review group and other people affected by Parkinson’s who provided feedback.
We’re the Parkinson’s support and research charity. Help us find a cure and improve life for everyone affected by Parkinson’s.

Can you help?
At Parkinson’s UK, we are totally dependent on donations from individuals and organisations to fund the work that we do. There are many ways that you can help us to support people with Parkinson’s. If you would like to get involved, please contact our Supporter Services team on 020 7932 1303 or visit our website at parkinsons.org.uk/support. Thank you.

Parkinson’s UK
Free* confidential helpline 0808 800 0303
Monday to Friday 9am–8pm, Saturday 10am–2pm. Interpreting available.
Text Relay 18001 0808 800 0303
(for textphone users only)
hello@parkinsons.org.uk
parkinsons.org.uk
*calls are free from UK landlines and most mobile networks.

Has this resource given you information that might help you manage your condition better?
☐ NA  ☐ It hasn’t helped  ☐ It has helped a little  ☐ It has helped a lot
What aspects did you find most helpful?
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Were you looking for any information that wasn’t covered?
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Do you have any other comments?
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If you would like to become a member of Parkinson’s UK, or are interested in joining our information review group, please complete the details below and we’ll be in touch.
☐ Membership  ☐ Information review group (who give us feedback on new and updated resources)
Name ...............................................................................................................................................................................................
Address ........................................................................................................................................................................................................
Telephone ........................................................................................................... Email .............................................................................................................
What is your ethnic background?  ☐ Asian or Asian British  ☐ Black or Black British  ☐ Chinese  ☐ Mixed
☐ White British  ☐ White other  ☐ Other (please specify).....................................................................................................................