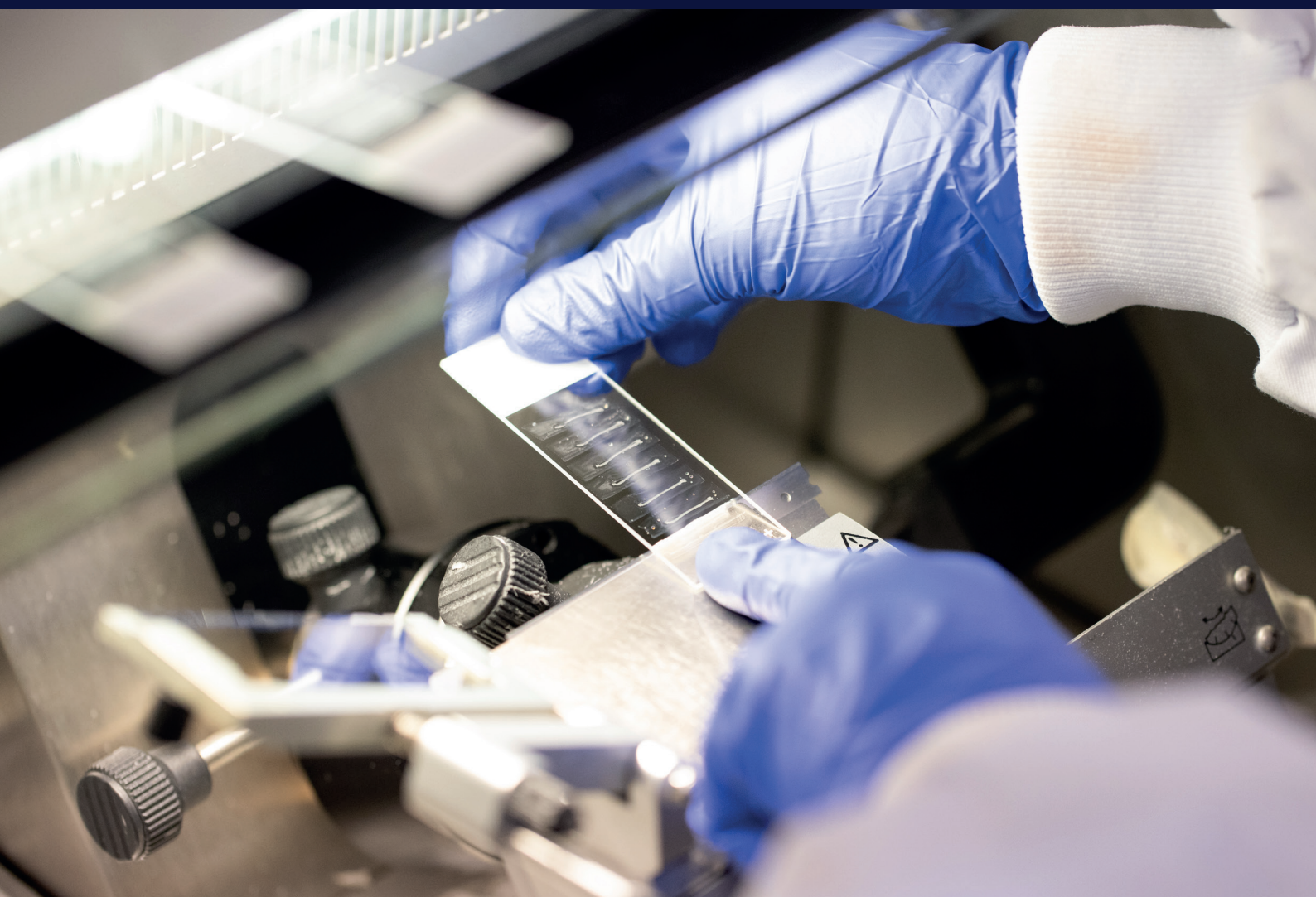


# PARKINSON'S VIRTUAL BIOTECH PORTFOLIO

Parkinson's  
Virtual Biotech

November 2024

Next update: May 2025



**PARKINSON'S<sup>UK</sup>**  
CHANGE ATTITUDES.  
FIND A CURE.  
JOIN US.

 **Parkinson's**  
Foundation

# ACTIVE PROJECTS

November 2024

Parkinson's  
Virtual Biotech

A groundbreaking global movement to deliver life-changing new treatments in years not decades.

We believe new and better treatments for Parkinson's are possible, but there is simply not enough focus or investment in turning exciting discoveries into new therapies. That's where the Parkinson's Virtual Biotech comes in. We designed the programme to bridge this gap and help take promising ideas for new therapies forward.

The Parkinson's Virtual Biotech uses the methods of the biotech world and venture funding to invest in drug development, but with the Parkinson's community at the heart of the decisions.

Founded by Parkinson's UK in 2017, the Parkinson's Virtual Biotech is now an international programme in partnership with the Parkinson's Foundation. We believe we'll get to a cure faster by collaborating, not competing.

We identify projects with the greatest potential to transform life for people with Parkinson's. We then work with partners to rapidly develop and test them. We have committed over £30 million towards our Virtual Biotech programme.

## Types of research project



**Cure projects** work towards treatments and strategies to slow, stop, reverse or prevent Parkinson's. This includes developing new treatments, and improving diagnosis and monitoring of the condition. We are currently funding 10 projects that fit into the Cure category through the Virtual Biotech.



**Life projects** work towards treatments and strategies to improve the symptoms and quality of life of people with Parkinson's. This includes better therapies to manage specific symptoms of Parkinson's. We are currently funding two projects that fit into the Life category through the Virtual Biotech.

## Stages of the research pipeline



### Scientific discoveries

Researchers attempt to find out what goes wrong in Parkinson's and come up with ideas for how to fix it.



### Developing treatments

Dedicated teams turn the most promising scientific discoveries into potential new treatments.



### Clinical trials

New treatments that have been proven safe and effective by all other methods are carefully tested in volunteers.



# ACTIVE PROJECTS



## November 2024



Our innovative approach is working. The next treatment is closer than ever.



Here are some of the latest projects we're investing in:

<b>Project name: Keapstone</b>	<b>Developing drugs to target oxidative stress</b>
<b>Investment committed to date</b>	£2.78m
<b>Type: Cure   Stage: Developing treatments</b>  	
<p>Keapstone is a company co-founded by researchers at the University of Sheffield and Parkinson's UK. It's looking at developing drugs that act on multiple pathways believed to be important in the development of Parkinson's. Recent findings have provided interesting avenues for further studies. Keapstone has grown out of the first project the Parkinson's Virtual Biotech took on in 2017.</p> <p>We are now providing new investment to develop these results to further investigate a molecule that has the potential to protect brain cells in lab experiments. The effectiveness and safety of the molecule will be assessed to inform whether clinical trials could take place in the future.</p>	



<b>Project name: CBD (CAN-PDP)</b>	<b>Clinical trial to investigate cannabidiol (CBD) for Parkinson's-related psychosis</b>
<b>Investment committed to date</b>	£1.52m
<b>Type: Life   Stage: Clinical trials</b>  	
<p>There are many different symptoms of Parkinson's and not everyone will experience the same ones. Evidence shows that up to 75% of people with Parkinson's go on to develop symptoms of hallucinations or delusions as their condition progresses. In October 2019, we announced we're partnering with researchers at King's College London to carry out a clinical trial to see whether CBD is safe and effective for treating symptoms of hallucinations or delusions in Parkinson's. The first stage of the study, a six-week pilot to find the ideal dosage of oral CBD capsules, is now complete.</p> <p>In 2023, the second stage of the trial started recruitment, involving 120 people with Parkinson's who experience problems with these symptoms taking part in a 12-week, double-blind, placebo-controlled study – the gold standard for testing new treatments.</p>	



<b>Project name: TOP HAT</b>	<b>A phase 2 clinical trial to explore the potential of ondansetron for treating hallucinations in people with Parkinson's or Lewy body dementia</b>
<b>Investment committed to date</b>	£1.26m
<b>Type: Life   Stage: Clinical trials</b>  	
<p>In October 2020, we announced our partnership with University College London to explore the potential of ondansetron as a treatment for visual hallucinations in people with Parkinson's or Lewy body dementia.</p> <p>Ondansetron is currently used to treat sickness following operations or during chemotherapy. It is estimated that around 75% of people with Parkinson's experience visual hallucinations, when they see things that aren't really there, during the course of their condition. These symptoms can be extremely distressing for people with Parkinson's and their families. However, current treatment options are limited.</p> <p>This study is investigating whether ondansetron is beneficial and safe as a treatment for hallucinations in up to 306 people with Parkinson's or Lewy body dementia.</p>	



<b>Project name: NRG</b>	<b>Targeting brain cell batteries to slow the progression of Parkinson's</b>
<b>Investment committed to date</b>	£5m
<b>Type: Cure   Stage: Developing treatments</b>  	
<p>In July 2019, we announced our partnership with NRG Therapeutics Ltd to find ways to boost the functioning of mitochondria in Parkinson's.</p> <p>Mitochondria, the powerhouses of the cell, play an important role in both sporadic and inherited forms of Parkinson's. With the help of the initial Virtual Biotech investment, NRG Therapeutics Ltd designed small molecules that are able to pass across the blood brain barrier and patch up a hole in the mitochondria wall to help prevent the loss of energy and cell death.</p> <p>Building on the success of the project, in 2022 NRG secured funding worth £16m, including further investment from the Parkinson's Virtual Biotech. This funding will be used to continue developing these molecules in animal models and progress them towards clinical trials. This offers hope as a potential way to protect mitochondria and help stop brain cells from dying.</p>	



<b>Project name: EndLyz</b>	<b>Finding ways to boost cell recycling in Parkinson's</b>
<b>Investment committed to date</b>	£980,000
<b>Type: Cure   Stage: Developing treatments</b>  	
<p>We're working with EndLyz Therapeutics, Inc. to help find therapeutic ways to clear cells of damaging or unwanted materials that might contribute to the causes of Parkinson's.</p> <p>Recent research suggests that lysosomes, packets of digestive chemicals that help to break down and recycle unwanted material inside cells, may be central to the development and progression of Parkinson's. When lysosomes don't work properly, brain cells can't get rid of old and damaged proteins, so these build up and clump together, slowly clogging up cells.</p> <p>This project will focus on developing new therapies to restore efficient lysosomal function, which may have the potential to slow or stop Parkinson's.</p>	



<b>Project name: Ambroxol</b>	<b>A phase 3 clinical trial investigating the potential of ambroxol for slowing down the progression of Parkinson's</b>
<b>Investment committed to date</b>	£1.1m
<b>Type: Cure   Stage: Clinical trials</b>  	
<p>Parkinson's UK is partnering with research charity Cure Parkinson's, Van Andel Institute and John Black Charitable Foundation to co-fund a trial looking at the potential of ambroxol, a drug found in a cough medicine which has been used for many years, to slow the progression of Parkinson's.</p> <p>The ASPro-PD trial is a world-first phase 3 trial of ambroxol. Driven by Cure Parkinson's, following 8 years of work with the Parkinson's community, this £5.5m trial offers hope that a drug to slow the progression of Parkinson's may be on the horizon. Results from phase 2 of the clinical trial show that ambroxol increases a protein called GCase, which helps break down and remove waste proteins, such as toxic alpha-synuclein from cells. This is the first large phase 3 study the Parkinson's Virtual Biotech has funded.</p>	



<b>Project name:</b> Syntara (previously known as Pharmaxis)	A phase 2 clinical trial of a new treatment that aims to relieve Parkinson's-like symptoms and target inflammation to slow the onset of the condition
<b>Investment committed to date</b>	£2.9m
<b>Type:</b> Cure   <b>Stage:</b> Clinical trials  	
<p>In September 2022, we announced we're working with Syntara to investigate whether a drug called PXS-4728 can reduce inflammation in the very early stages of Parkinson's.</p> <p>Inflammation is part of the body's natural response to injury, but it can cause problems if it is overactive and actually damages cells. This is thought to contribute to the causes and progression of Parkinson's.</p> <p>This study will investigate PXS-4728 in 40 people who experience a sleep disorder known as isolated rapid eye movement sleep behaviour disorder (iRBD).</p> <p>Studies suggest as many as 70% of people with iRBD go on to develop Parkinson's. The hope is that this drug might be able to slow the onset of Parkinson's symptoms in this group of people that are at a high risk of developing the condition. This could help find a way to slow the progression of Parkinson's in others with the condition. The first participant was recruited in November 2023.</p>	

<b>Project name:</b> Neumora	Drug development to target inflammation in the brain
<b>Investment committed to date</b>	£2.1m
<b>Type:</b> Cure   <b>Stage:</b> Developing treatments  	
<p>Research shows that there is more inflammation in the areas of the brain affected by Parkinson's. This is thought to play a potential role in damaging the dopamine-producing cells in the brain, causing Parkinson's to progress faster.</p> <p>We have partnered with Neumora Therapeutics Inc., a US-based company, to help fund and accelerate the final lab-based research needed to advance a potential new drug that targets inflammation. The drug aims to protect brain cells affected by Parkinson's by stopping inflammation being triggered in the brain.</p> <p>If this two-year project is successful, the drug will be ready to move towards clinical trials involving people with Parkinson's.</p>	

<b>Project name: Mission</b>	<b>First clinical trial of MTX325 in people with Parkinson's</b>
<b>Investment committed to date</b>	£1.25m
<b>Type: Cure   Stage: Clinical trials</b>	
 	
<p>Mitochondria are small structures responsible for producing the energy cells need to function properly, including brain cells. In Parkinson's, mitochondria inside dopamine-producing brain cells stop working properly.</p> <p>MTX325 is a compound that has been designed to help get rid of faulty mitochondria. By removing 'problem mitochondria' the team hope to protect dopamine-producing brain cells and ultimately slow or even stop the progression of the condition. After successful studies in the lab, MTX325 is currently being trialled in healthy individuals to understand its safety.</p> <p>In July 2024, we announced a partnership with Mission Therapeutics and the Michael J. Fox Foundation for Parkinson's Research to fund the next stage of this research – investigating the safety and benefits of the potential drug in people with Parkinson's.</p>	

<b>Project name: Lucy</b>	<b>Development of potential new treatments to protect brain cells using mitochondria</b>
<b>Investment committed to date</b>	£1.6m
<b>Type: Cure   Stage: Developing treatments</b>	
 	
<p>In June 2024, we announced a partnership with Lucy Therapeutics to drive forward research looking at new drugs to restore mitochondria. Mitochondria are responsible for producing the energy cells need to function properly.</p> <p>Lucy Therapeutics has developed molecules that early experiments show can target a key part of the mitochondria and improve its function. With investment from this partnership, they will look to see in more detail how the molecules might be able to protect brain cells. This will involve exploring whether the molecules can reduce clumps of a protein thought to damage brain cells. This testing should hopefully improve understanding of how the new drug could work, and will bring it closer towards the next stage of testing in clinical trials.</p>	

<b>Project name: Herantis</b>	<b>First clinical trial of a pioneering new treatment, HER-096, in people with Parkinson's</b>
<b>Investment committed to date</b>	£1.55m
<b>Type: Cure   Stage: Clinical trials</b>	
 	
<p>HER-096 is a new treatment that aims to protect and restore the dopamine-producing brain cells that are lost in Parkinson's. It has been developed based on a growth factor the brain naturally produces, called CDNF (Cerebral Dopamine Neurotrophic Factor).</p> <p>Research in the lab shows CDNF can help damaged dopamine cells to survive and recover. However, CDNF is a large protein that can't easily cross from the blood into the brain, so giving it to patients would require complex and invasive surgery. Herantis Pharma has developed a compound called HER-096 that has similar properties to CDNF but is smaller, can access the brain and be given as a simple injection.</p> <p>HER-096 has already been tested in a study with 60 healthy participants and was shown to be safe, with no serious side effects. This investment from the Parkinson's Virtual Biotech and Michael J. Fox Foundation will fund a new study to test if regular injections of HER-096 are safe in people with Parkinson's. The study is now underway in Finland.</p>	



<b>Project name: Acurex</b>	<b>Fine tuning CU-13001 to progress it towards clinical trials for Parkinson's</b>
<b>Investment committed to date</b>	£1.59m
<b>Type: Cure   Stage: Developing treatments</b>	
 	
<p>We're investing £1.59m through our Virtual Biotech programme to drive forward the development of a promising molecule which has potential to become a drug that slows or stops Parkinson's. Partnering with US-based company Acurex Biosciences, we are supporting the final stages of testing and development of their molecule, CU-13001, in preparation for its progress into clinical trials.</p> <p>CU-13001 is a molecule that has shown exciting potential for protecting the brain cells that are lost in Parkinson's in laboratory-based tests. It targets an enzyme called 15-lipoxygenase, which emerging research suggests may play an important role in the death of dopamine-producing cells in the condition.</p>	








# PAST PROJECTS



## November 2024



The Virtual Biotech programme is dynamic and agile – new investments are added and other investments come to an end. There are multiple reasons projects move from ‘active’ to ‘past’ – it could be because they are seeking other funding to continue or a decision has been made to stop an investment because the project is not meeting its aims. This is a vital part of ensuring the most promising research is moving forward as quickly as possible.



<b>Project name:</b> Neurolixis	<b>A phase 2 trial of the drug NLX-112 for treating dyskinesia in people with Parkinson’s</b>
<b>Investment committed to date</b>	£1.57m
<b>Type:</b> Life   <b>Stage:</b> Clinical trials  	
<p>Since 2017, we’ve been working with biopharmaceutical company Neurolixis to investigate a drug called NLX-112 for the treatment of involuntary movements (dyskinesia). This is a common side effect experienced by people with Parkinson’s who have been taking levodopa-based medications for several years. These studies investigated NLX-112 in the lab and secured approval from regulatory authorities to take the drug into clinical trials in people with Parkinson’s.</p> <p>In November 2020, we announced we’d be funding a new phase 2a trial in 22 people with Parkinson’s. Results show the drug was safe and was beneficial for those who experience dyskinesia. The trial also showed that the drug had a positive effect on other motor symptoms of Parkinson’s. This is an early-stage trial, and its main aim was to prove the safety and understand how the body reacts to the drug, so that it can be tested on a larger scale. Neurolixis is currently planning how to best take this exciting project forward to understand the effectiveness of the drug in more people and over a longer period of time.</p>	

<b>Project name:</b> Eurofins	<b>Creating new drugs to improve symptoms and slow Parkinson’s</b>
<b>Investment committed to date</b>	£2.83m
<b>Type:</b> Cure/Life   <b>Stage:</b> Developing treatments   	
<p>Back in March 2018, we announced that we would be collaborating with one of the UK’s leading contract research companies, Selcia (now known as Eurofins), aiming to create new molecules to increase the activity of a selection of genes to both increase dopamine production, and boost the production of protective proteins to slow or halt the damage and loss of precious brain cells in Parkinson’s.</p> <p>The project did not meet its milestones to find potential molecules to take forward. There is still potential for this work but after independent review the Virtual Biotech will not be investing further at this time.</p>	

<b>Project name:</b> Sheffield	<b>Discovering molecules that restore brain cell batteries</b>
<b>Investment committed to date</b>	£1.3m* (we expect to see return of some funding)
<b>Type:</b> Cure   <b>Stage:</b> Developing treatments  	
<p>Through our partnership with Sheffield University, drug-like molecules have been tested to see if they can help protect brain cells and boost the function of mitochondria, the batteries of the cell. As we progressed with the project, we made new discoveries that provided valuable insights.</p> <p>However, it became clear that the original project plan was too limited to enable the delivery of promising drug candidates. So we have decided to hand the project back to Sheffield for further scientific exploration and have ceased Virtual Biotech funding at this point. We believe this decision will facilitate the advancement of the research in a more suitable academic environment at this stage.</p>	

<b>Project name:</b> Galaxy	<b>Finding ways to dial down inflammation in Parkinson's</b>
<b>Investment committed to date</b>	£2.4m
<b>Type:</b> Cure   <b>Stage:</b> Developing treatments  	
<p>Inflammation is a vital process for defending the body against harm from things like infections, injuries and toxins. It should only be activated when there is a threat. If inflammation is active when it shouldn't be, it can cause harm to healthy cells. There is increasing evidence that this might be the case in Parkinson's. Announced in December 2021, this project looked to uncover ways to dial down inflammation in the brain to protect brain cells.</p> <p>The project has been stopped because it looked increasingly unlikely to produce a new treatment.</p>	

<b>Project name:</b> Vivifi GDNF	<b>Planning a new clinical trial of device-delivered GDNF</b>
<b>Investment committed to date</b>	£1.2m* (Final amount to be confirmed)
<b>Type:</b> Cure   <b>Stage:</b> Clinical trials	
 	
<p>GDNF (glial cell-derived neurotrophic factor) is a special protein that is naturally produced inside the brain. When GDNF is given to damaged dopamine brain cells in the lab it helps them to regenerate. It may be able to do the same in people with Parkinson's if it can be delivered to the right part of the brain in the right way.</p> <p>This project looked at finding a way forward for a clinical trial of device delivered GDNF that has potential to help slow, stop or reverse the progression of Parkinson's. We have decided not to continue investing in device delivered GDNF as the costs and difficulties became too high. There are other trials in progress that continue to investigate the potential of GDNF. We are also now funding a clinical trial of an injectable form of a growth factor that has similar potential to GDNF.</p>	

<b>Project name:</b> Enterin	<b>Investigating a new treatment for Parkinson's-related dementia</b>
<b>Investment committed to date</b>	£0
<b>Type:</b> Life   <b>Stage:</b> Clinical trials	
 	
<p>A trial exploring the potential of a drug called ENT-01 to address Parkinson's dementia, supported by Parkinson's UK, is no longer going ahead.</p> <p>ENT-01 has the potential to reduce the effects of alpha-synuclein, a protein thought to play an important role in the development of Parkinson's, in the gut. Early-stage clinical trials in people with Parkinson's suggest the drug improves constipation and revealed it may hold promise for treating other important aspects of Parkinson's, including dementia.</p> <p>We announced £2m of funding for the proposed trial through our Parkinson's Virtual Biotech programme in October 2022. The total cost of the trial was anticipated to be over £10m. The company Enterin Inc. aimed to raise this from other investors to start the study in early 2024.</p> <p>Unfortunately, despite their best efforts, the company has not been able to raise enough investment to take the study forward at this time. However, Enterin remains fully committed to developing ENT-01 for Parkinson's constipation.</p>	

We are Parkinson's UK.  
Powered by people.  
Funded by you.  
Improving life for everyone  
affected by Parkinson's.  
Together we'll find a cure.

**PARKINSON'S<sup>UK</sup>**  
**CHANGE ATTITUDES.**  
**FIND A CURE.**  
**JOIN US.**

Parkinson's  
Virtual Biotech

Free confidential helpline **0808 800 0303**  
Monday to Friday 9am to 6pm, Saturday 10am to 2pm  
(interpreting available)  
NGT relay **18001 0808 800 0303**  
(for textphone users only)  
**hello@parkinsons.org.uk**  
**parkinsons.org.uk**

Parkinson's UK, 50 Broadway, London, SW1H 0DB

Last updated November 2024 next update due May 2025.

Parkinson's UK is the operating name of the Parkinson's Disease Society of the United Kingdom.  
A charity registered in England and Wales (258197) and in Scotland (SC037554). © Parkinson's UK 10/24 (244109)