ACTIVE PROJECTS

Parkinson's Virtual Biotech

May 2024
Next update: November 2024

PARKINSON'S UK
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Parkinson's Foundation

ACTIVE PROJECTS
May 2024

Parkinson's Virtual Biotech

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

Like other biotechs, the Parkinson's Virtual Biotech uses cutting edge biological and chemical research to come up with new treatments. But it's driven by people with Parkinson's, not profit. Collaborative and agile, it adapts successful methods from the business world to deliver new treatments faster.

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.

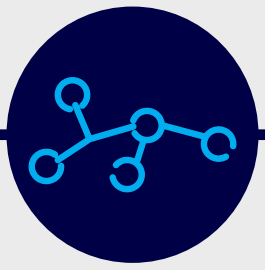
Types of research project

Cure (disease modifying) 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.

Life (symptomatic) projects work towards treatments and strategies to improve the symptoms and quality of life of people with Parkinson's. This includes better therapies and management for issues such as dyskinesia, hallucinations, and thinking and memory problems.

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.

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

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

Project name: Keapstone	Developing drugs to target oxidative stress (I-1701)
Investment approved to date	£3.31m
Type: Cure | **Stage:** Developing treatments 	
Keapstone is a company co-founded by researchers at the University of Sheffield and Parkinson's UK. This project was the first to be taken on by the Parkinson's Virtual Biotech in 2017. The work is looking at developing drugs that act on multiple pathways which are believed to be important for the development of Parkinson's. Recent findings have provided interesting avenues for further studies, and we are now providing a new investment to develop these results.	

Project name: Eurofins	Creating new drugs to improve symptoms and slow Parkinson's (I-1703)
Investment approved to date	£2.8m
Type: Cure/Life | **Stage:** Developing treatments 	
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), to create new molecules that can increase the activity of a selection of genes. Dialling up the activity of these genes has the potential both to increase dopamine production, and boost the production of protective proteins to slow or halt the damage and loss of precious brain cells. If we're successful, it could lay the foundations for research into new treatments that could not only improve Parkinson's symptoms, but also slow, stop or even reverse the underlying condition.	

Project name: CBD (CAN-PDP)	Clinical trial to investigate cannabidiol (CBD) for Parkinson's-related psychosis (I-1901)
Investment approved to date	£1.52m

Type: Life | **Stage:** Clinical trials

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.

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.

Project name: TOP HAT	A phase 2 clinical trial to explore the potential of ondansetron for treating hallucinations in people with Parkinson's or Lewy body dementia (I-1902)
Investment approved to date	£1.26m

Type: Life | **Stage:** Clinical trials

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.

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.

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. Following an interim analysis, TOP HAT is no longer open to recruitment.

Project name: NRG	Targeting brain cell batteries to slow the progression of Parkinson's (I-1903)
Investment approved to date	£4.5m

Type: Cure | **Stage:** Developing treatments

In July 2019, we announced our partnership with NRG Therapeutics Ltd to find ways to boost the functioning of mitochondria in Parkinson's.

Mitochondria, the powerhouses of the cell, play an important role in both sporadic and inherited forms of Parkinson's. The aim of this project is to identify new molecules that can enter the brain and support the mitochondria.

If successful, these protective molecules could provide a safe and effective new treatment that will protect brain cells, slow the progression of Parkinson's and extend quality of life.

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 and progress towards clinical trial.

Project name: Sheffield	Discovering molecules that restore brain cell batteries (I-1904)
Investment approved to date	£1.3m

Type: Cure | **Stage:** Developing treatments

We're partnering with researchers at the University of Sheffield to discover molecules that can boost the function of brain cell batteries. This research aims to take important steps towards creating a drug that can protect dopamine-producing brain cells and slow down the progression of Parkinson's. The team is currently developing drug-like molecules which will be tested in cells from people with Parkinson's.

Project name: Galaxy	Finding ways to dial down inflammation in Parkinson's (I-2001)
Investment approved to date	£3.1m

Type: Cure | **Stage:** Developing treatments 

Announced in December 2021, this project aims to find a way to stop harmful inflammation from damaging brain cells.

Inflammation is a process that is vital 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.

This project looks to uncover a way to dial down inflammation in the brain, in the hope to protect brain cells. This could help pave the way for the design of a drug to help slow or stop the condition.

Project name: EndLyz	Finding ways to boost cell recycling in Parkinson's (I-2102)
Investment approved to date	£905,000

Type: Cure | **Stage:** Developing treatments 

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.

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 choking cells.

This project will focus on developing new therapies to restore efficient lysosomal function, which may have the potential to slow or stop Parkinson's.

Project name: Ambroxol	A phase 3 clinical trial investigating the potential of ambroxol for slowing down the progression of Parkinson's (I-2202)
Investment approved to date	£1.1m

Type: Cure | **Stage:** Clinical trials

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.

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.

Project name: 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 (I-2201)
Investment approved to date	£2.9m

Type: Cure/Life | **Stage:** Clinical trials

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.

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.

This study will investigate PXS-4728 in 40 people who experience a sleep disorder known as isolated rapid eye movement sleep behaviour disorder (iRBD).

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.

Project name: Neumora	Drug development to target inflammation in the brain
Investment approved to date	£2.1m

Type: Cure | **Stage:** Developing treatments

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.

We've 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.

If this two-year project is successful, the drug will be ready to move towards clinical trials involving people with Parkinson's.

Project name: Neurolixis	A phase 2 trial of the drug NLX-112 for treating dyskinesia in people with Parkinson's (I-2002)
Investment approved to date	£1.57m

Type: Life | **Stage:** Clinical trials

Since 2017, we've been working with biopharmaceutical company Neurolixis and US charity The Michael J. Fox Foundation (MJFF) 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.

In November 2020, we announced we'd be funding a new phase 2a trial leading on from these previous studies funded by the MJFF and Parkinson's UK. Results announced in July 2023 show the drug is safe to use for people with Parkinson's and it is beneficial for those who experience dyskinesia. The trial also showed that the drug had a positive effect on other motor symptoms of Parkinson's, such as slowness, stiffness and tremor.

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. The next stage of the trial will aim to find out the best and safest doses and gather more information on the effectiveness with more participants over a longer period. Neurolixis is currently planning how to best take this exciting project forward to the next stage of clinical trials.

If the next phases are successful, the drug could be approved and available by 2030.

Project name: GDNF	Project complete: Planning a new clinical trial of device-delivered GDNF (I-2101)
Investment approved to date	£800,000

Type: Cure | **Stage:** Clinical trials

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.

Clinical trials using different devices to deliver GDNF have given inconclusive results, but we believe that GDNF still holds huge promise for people with Parkinson's.

In 2019 we launched a new company, Vivifi Biotech, to look at finding a way forward for a clinical trial of device delivered GDNF to further investigate its potential to help slow, stop or reverse the progression of Parkinson's.

We've reviewed the scientific evidence from the first trial and listened to the views of people with Parkinson's about whether it is right to move ahead. The decision has been made not to continue investing in device delivered GDNF as there may be other opportunities for alternative ways to investigate the potential of GDNF for Parkinson's.

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.

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