

# The LITE study: shining LIGHT on Parkinson's disease

<b>Submission date</b> 13/06/2025	<b>Recruitment status</b> Recruiting	<input checked="" type="checkbox"/> Prospectively registered <input type="checkbox"/> Protocol
<b>Registration date</b> 17/06/2025	<b>Overall study status</b> Ongoing	<input type="checkbox"/> Statistical analysis plan <input type="checkbox"/> Results
<b>Last Edited</b> 16/07/2025	<b>Condition category</b> Nervous System Diseases	<input type="checkbox"/> Individual participant data <input checked="" type="checkbox"/> Record updated in last year

## Plain English summary of protocol

### Background and study aims

Scientists are still working to understand fully why Parkinson's disease (PD) happens. It is likely to be due to, or at least in part due to, a combination of genetic and environmental factors as well as ageing. There is no test for diagnosing PD, for measuring how it progresses, or predicting what the outcome for people living with PD will be. As new approaches to treatments are being tested in clinical trials, we don't know whether these new treatments might help all people with PD or only a smaller group of people with PD.

The LITE study is important as it will help to fill these critical gaps in our knowledge. The LITE study is especially important as it will be collecting a lot of biological samples, imaging data and information from a large group of people, with and without PD. Scientists will be able to look at all the information we collect, and their work will add to our general knowledge about PD

### Who can participate?

Patients aged 18 years and over with PD, and healthy volunteers

### What does the study involve?

You will be invited to come to the Ninewells Hospital, Dundee (or one of the global sites) to have biological samples taken, a Magnetic Resonance Imaging scan (MRI) brain scan, a dopamine transporter (DaT) SPECT brain scan and assessments of PD signs and symptoms (even if you don't have PD). Details of all the assessments are given below.

Before you have any assessments, we will ask you to complete a consent form to say that you want to take part in the study. You will be able to speak to a study doctor before you sign the consent form if you want.

You will have all these assessments whether or not you have PD: review of your medical history, medications and treatments, family history, ethnicity, physical and neurological examination, smell identification test, questionnaires, blood samples, urine sample, sebum sample, breath sample, skin biopsies, cerebrospinal fluid sample, MRI scan and DaT scan.

### What are the possible benefits and risks of participating?

The main benefit of taking part will be that you will be contributing to the understanding of PD and eventually the possible development of new treatments which could benefit you and future patients. The study may not immediately benefit you, but if the results of the study are positive

this may improve how we treat people with PD.

There are a lot of assessments for you to complete for the study and this may be tiring for you. We can arrange for the assessments to be split over 2 or more days, so you don't get too tired. You can ask your study team to stop the assessments at any time and come back another day to finish them. We can also arrange the visits so that you have a "rest day" between visits. All of the medical procedures outlined in the PIS have an element of risk and this is detailed further in the information sheet. Your study doctor will discuss these in detail with you ahead of participating and during the informed consent process.

Where is the study run from?

The study is led by the University of Dundee at Ninewells Hospital. This will be the only UK site. There will be several other sites globally.

When is the study starting and how long is it expected to run for?

October 2024 to August 2027

Who is funding the study?

Michael J. Fox Foundation for Parkinson's Research (USA)

Who is the main contact?

Dr Esther Sammler, e.m.sammler@dundee.ac.uk

## Contact information

### Type(s)

Scientific, Principal investigator

### Contact name

Dr Esther Sammler

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Public, Scientific

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### **Type(s)**

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### **Contact name**

Miss Tiffany Stewart

### **Contact details**

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## **Additional identifiers**

### **Clinical Trials Information System (CTIS)**

Nil known

### **Integrated Research Application System (IRAS)**

351205

### **ClinicalTrials.gov (NCT)**

Nil known

### **Protocol serial number**

CPMS 66112

## **Study information**

### **Scientific Title**

The MJFF LRRK2 Investigative Therapeutics Exchange (LITE) Study: the LITE study

### **Acronym**

MJFF:LITE

### **Study objectives**

The proposed LITE study is the translational pillar of the LITE Initiative and is designed as an observational, multi-center international study with the additional option for follow-up. The LITE study aims to discover and test new biological markers for LRRK2-driven Parkinson's disease (PD) in various genetic (monogenic) PD and non-genetic (idiopathic) PD populations, people at risk for PD and healthy controls and correlate these with clinical and imaging features of disease state and progression.

### **Ethics approval required**

Ethics approval required

### **Ethics approval(s)**

approved 21/05/2025, East of Scotland Research Ethics Service (Tayside Medical Science Centre, Residency Block, Level 3, George Pirie Way, Ninewells Hospital and Medical School, Dundee, DD1 9SY, United Kingdom; +44 (0)1382 383900; tay.eosres@nhs.scot), ref: 25/ES/0023

### **Study design**

Cross-sectional observational multi-center international study

### **Primary study design**

Observational

### **Study type(s)**

Other

### **Health condition(s) or problem(s) studied**

Parkinson's disease

### **Interventions**

Participants will undergo clinical (motor, neuropsychiatric and cognitive) and imaging assessments and will donate biological samples including blood (e.g. plasma, Peripheral blood mononuclear cells (PBMCs), neutrophils and monocytes), urine, skin biopsies and skin swabs, breath and CSF for comprehensive biological sample analysis. Every participant will also undergo genetic testing for PD-relevant genes.

### **Intervention Type**

Other

### **Primary outcome(s)**

1. Lysosomal lipids, metabolites and proteins in enriched lysosomal fractions from peripheral blood immune cells, measured using targeted and untargeted mass spectrometry in tagless LysoIP samples isolated from peripheral blood mononuclear cells (PBMCs) and monocytes at a single cross-sectional timepoint
2. LRRK2 levels and LRRK2 kinase activity in peripheral blood immune cells, measured using a targeted mass spectrometry assay in peripheral blood monocytes and neutrophils
3. Presence of misfolded, aggregation-prone alpha-synuclein species, measured using an alpha-synuclein seed amplification assay in cerebrospinal fluid and in skin homogenates collected at the study visit

### **Key secondary outcome(s)**

1. BMP (bis(monoacylglycerol)phosphate) levels in urine, measured using targeted mass spectrometry from urine samples collected at a single cross-sectional timepoint
2. Mass-spectrometry profiles in cerebrospinal fluid (CSF), measured using data-independent acquisition (DIA) mass spectrometry from CSF collected at the study visit
3. Mass-spectrometry profiles in plasma, measured using high-resolution mass spectrometry-based lipidomics from plasma collected at a single cross-sectional timepoint
4. Mass-spectrometry profiles in urine, measured using untargeted ultra-high performance liquid chromatography – mass spectrometry (UHPLC-MS) from urine samples collected at the study visit
5. Genetic variants associated with Parkinson's disease, measured using next-generation sequencing (NGS) from genomic DNA isolated from whole blood at the study visit

## **Completion date**

31/08/2027

## **Eligibility**

### **Key inclusion criteria**

Parkinson's disease group:

1. Age  $\geq$ 18 years
2. Willingness to undergo genetic testing
3. Able to provide informed consent
4. Diagnosis of PD meeting the Movement Disorder Society Clinical Diagnostic Criteria for clinically established PD
5. Bradykinesia and resting tremor or rigidity

Additional inclusion criteria for PD subgroups:

1. Genetic PD: confirmation of carrier status of a pathogenic or probable pathogenic variant in LRRK2, VPS35, Rab32, GBA1 or other monogenic form of PD.
2. Non-genetic PD (Idiopathic PD): absence of carrier status of a pathogenic or probable pathogenic variant in LRRK2, VPS35, Rab32, GBA1 or other monogenic form of PD.

Non-Parkinson's disease group:

1. Age  $\geq$ 18 years
2. Willingness to undergo genetic testing
3. Able to provide informed consent

Additional inclusion criteria for non-PD subgroups:

1. No PD and no genetic carrier status for PD ('healthy controls')
2. Absence of carrier status of a pathogenic or probable pathogenic variant in LRRK2, VPS35, Rab32, GBA1 or in any other clearly PD-related gene
3. Absence of a first-degree relative with PD (e.g., biological parent, sibling, child) or multi-incident family history of PD

No PD but non-manifesting pathogenic variant carrier status:

1. Confirmation of carrier status of a pathogenic or probable pathogenic variant in LRRK2, VPS35, Rab32, GBA1 or other monogenic form of PD

No PD but positive family history of PD:

1. First-degree relative with PD (e.g., biological parent, sibling, child) or multi-incident family history of PD

2. Unknown genetic carrier status of a pathogenic or probable pathogenic variant in LRRK2, VPS35, Rab32, GBA1 or other monogenic form of PD

**Participant type(s)**

Healthy volunteer, Patient, Other

**Healthy volunteers allowed**

No

**Age group**

Adult

**Lower age limit**

18 years

**Sex**

All

**Key exclusion criteria**

Exclusion criteria for all participants with PD:

1. A clinical diagnosis of dementia as determined by the investigator
2. Clinical evidence of atypical Parkinsonism (e.g., multiple-system atrophy or progressive supranuclear palsy) or evidence of drug-induced Parkinsonism
3. Previously obtained Magnetic Resonance Imaging (MRI) scan with evidence of clinically significant neurological disorder (in the opinion of the Investigator)
4. Any other reason (medical or psychiatric condition or lab abnormality) that, in the opinion of the investigator, would render the participant unsuitable for study enrollment
5. Participation in a clinical trial testing an investigational medicinal product for PD that could interfere with the study investigations, e.g. LRRK2 or lysosome targeting IMPs (in the opinion of the investigator)

Exclusion criteria for MRI and DAT imaging with PD:

1. Pregnant, lactating or planning pregnancy during the study. Pregnancy test to be carried out on day of scan for women of childbearing potential. Iodine allergy is exclusionary

Exclusion criteria for all participants without PD:

1. Symptomatic PD syndromes due to either drugs (e.g., metoclopramide, flunarizine, neuroleptics) or metabolic disorders (e.g., Wilson's disease), encephalitis
2. Clinical evidence of atypical Parkinsonism (e.g., multiple-system atrophy or progressive supranuclear palsy)
3. A clinical diagnosis of dementia as determined by the investigator
4. Previously obtained Magnetic Resonance Imaging (MRI) scan with evidence of clinically significant neurological disorder (in the opinion of the Investigator)
5. Any other reason (medical or psychiatric condition or lab abnormality) that, in the opinion of the investigator, would render the participant unsuitable for study enrollment

Exclusion criteria for MRI and DAT imaging without PD:

1. Pregnant, lactating or planning pregnancy during the study. Pregnancy test to be carried out on day of scan for women of childbearing potential. Iodine allergy is exclusionary

**Date of first enrolment**

30/06/2025

**Date of final enrolment**

30/06/2027

## Locations

**Countries of recruitment**

United Kingdom

Scotland

**Study participating centre**

**Neuroprogressive and Dementia Network - NHS Tayside**

Clinical Research Centre

James Arrott Drive

Ninewells Hospital

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## Sponsor information

**Organisation**

University of Dundee

**ROR**

<https://ror.org/03h2bxq36>

## Funder(s)

**Funder type**

Charity

**Funder Name**

Michael J. Fox Foundation for Parkinson's Research

**Alternative Name(s)**

Michael J. Fox Foundation, Fundación Michael J. Fox, The Michael J. Fox Foundation for Parkinson's Research, The Michael J. Fox Foundation, Michael J Fox Foundation for Parkinson's Disease Research, Michael J Fox Foundation for Parkinson's Research, The Michael J Fox Foundation for Parkinson's Research, MJFF

**Funding Body Type**

Government organisation

**Funding Body Subtype**

Trusts, charities, foundations (both public and private)

**Location**

United States of America

## **Results and Publications**

**Individual participant data (IPD) sharing plan**

The datasets generated during and/or analysed during the current study will be stored in a non-publicly available repository (to be confirmed). The data sharing plan will be made available at a later date.

**IPD sharing plan summary**

Stored in non-publicly available repository