



Gaucher families helping find a cure for Parkinson's

# Help us with a pioneering new research study.

Study opens April 2016

[www.rapsodi.org.uk](http://www.rapsodi.org.uk)

## Gaucher-Parkinson's research link has 3 main goals

- To understand how the GBA gene can cause Parkinson's.
- To develop new and more accurate ways to predict Parkinson's years before any symptoms appear.
- To develop a drug to stop Parkinson's developing or progressing in those who have the gene variant.

Much work around the world is now being coordinated to achieve these goals. Doctors and scientists at the Royal Free Hospital, London are working with research groups from Italy, Canada, Germany, USA and Israel to study the link and are already testing potential new drugs to target the link.

**People with Gaucher and their families can play a crucial and leading role in new research to find clues to the cause and cure for Parkinson's.**

**RAPSODI** is an easy to use online study led by the neurology and haematology teams at the Royal Free Hospital and University College London. We are looking to find new treatments for Parkinson's disease leading ultimately to a cure. Latest research findings suggest that the Gaucher community is in a unique position to be able to offer help and special insights into this condition.

## Why can Gaucher help find answers for Parkinson's?

The genetic alteration around the GBA gene that causes Gaucher disease and is carried within Gaucher families has been providing new and interesting clues into the development of Parkinson's in some people. There is a slighter higher chance of developing Parkinson's in later life if you carry this genetic alteration, but this is still a very low risk and most people do not develop Parkinson's. Nevertheless this new discovery linking the GBA gene to Parkinson's is probably one of the most important discoveries in Parkinson's in recent years and opens up new and promising avenues for research into how we may target this Gaucher-Parkinson's (GD-PD) link and develop powerful new treatments for these diseases.



## How does the study work?

We want to follow people with the GBA gene over several years and so we've been working with people with Gaucher disease and their families to develop a study that is easy to use, doesn't take up too much time and won't involve hospital visits. The study is mostly internet based, so you can take part from your own home or somewhere local to where you live. We will also send you some tests by post which once again can be completed in your own home and sent back to us. The study plans to collect data we already know can predict certain aspects of Parkinson's many years before any symptoms appear.

## Predicting Parkinson's?

The overall aim of the study is not to predict each individual's risk of developing Parkinson's but to look for patterns in the bigger community which is why all data is kept anonymously. But by predicting Parkinson's earlier we will be able to offer help to everyone more at risk of developing the condition.

## Who is supporting and leading the study?



The study is sponsored by the Medical Research Council, the EU, Parkinson's UK, The Javon Charitable Trust and the Leonard Wolfson Experimental Neurology Centre. It is being led by Professor Anthony Schapira and his team in the Department of Clinical Neurosciences, UCL working with Professor Atul Mehta and Dr Derralynn Hughes and their team in the Department of Haematology at the Royal Free Hospital, London.

The study is supported by the Gauchers Association who we are working with to ensure the study is patient friendly, easy to participate in and that all information regarding the study and its progress is disseminated on a regular basis to participants and interested parties.

## Taking part involves 4 things



We would need you to join the online study and become a member of this GBA study group. You will be able to do this by visiting the study website and using a token we will provide you with to gain access to the study portal and your own unique membership login.



We will ask you some questions about your medical and family history, but all data will be confidentially stored, protected and encrypted with the same level of security used by the online banking industry.



Once a year we will ask you to take part in an online test that explores aspects of memory, sleep and some movement.



We will ask you to do a smell and saliva test that can be done at home and returned in the post.

## How to join the study?

If you are interested in joining the study or hearing more about what is involved please contact.

**Dr. Stephen Mullin or  
Sarah Cable**  
(Clinical Research Nurse)

**rapsodi@ucl.ac.uk  
s.cable@ucl.ac.uk**

**tel: 07753 982 063**

**Juniebel Cooke**  
(Clinical Nurse Specialist)

**juniebel.cooke@nhs.net**

And please don't underestimate your contribution and the difference you can make. Every bit of information helps us to get closer to finding valuable answers in understanding more about how Parkinson's develops and how we can stop this condition in its tracks.

**Thank you for your interest and for taking part.**