MND–SMART
Clinical trials for MND

THE UNIVERSITY of EDINBURGH
EUAN MACDONALD CENTRE
Vital research into motor neurone disease

Frequently asked questions

Taking part in the trial

Will I be able to find out which drug I'm taking?

No. This is a double-blind study, which means that neither the study team or trial participant know who is taking an active trial drug and who is taking a placebo. Drugs are specially packaged and labelled without their name to make sure that nobody involved in the research finds out who is taking what. If your doctor needs to know what trial drug you are taking, in the event of a medical emergency, they can find out.

Who can take part in MND-SMART?

Hundreds of people across the UK will be able to take part. The trial has been designed so that most people with MND will be eligible. People who are pregnant or breast-feeding and those who are already part of another drug trial will not be able to take part. There are some other criteria too, the trial team will discuss these with you

You can still take part in MND-SMART if you are taking Riluzole, receiving breathing support, NIV, or have a feeding tube.

Does how long I've been diagnosed with MND affect whether I can take part?

No, how long you have been diagnosed with MND doesn't affect whether you can join the trial.

Can people from outside the UK take part?

MND-SMART is only open to people with MND living and receiving their MND treatment in the UK.

What will taking part involve?

You will take a liquid medicine once a day. We will ask you to keep a diary to record when you took the drug, and any side effects you experienced. You will also need to attend appointments. Some appointments will be at your trial centre and the rest can be done over telephone or video conference.

How often will I have to attend appointments?

There are several weekly appointments early on whilst the dose of medicine is increased safely,, but later appointments are only every 2 months. Some appointments will be at the participating trial centre nearest to you, and the rest can take place over the phone or by secure video calls.

Your involvement could be anything from a few weeks up to a maximum of about 7 years. This depends on when you join the study and whether the trial data shows if a drug has any benefit for people living with MND.

The benefit of this is that participants won't take ineffective drugs for longer than they need to.

What tests do I have to take at appointments?

The tests and assessments vary between different appointments. These will include questionnaires about physical functioning, your mental health and your MND, blood tests, an ECG (heart trace), breathing tests, a cognitive (thinking, learning and memory) assessment, and a pregnancy test if relevant.

What happens if I join the trial but later change my mind?

You are free to stop participating in MND-SMART at any time without giving a reason. Your decision will not affect the medical care you receive. The trial team will discuss with you how to safely reduce and stop taking the medicine.

Will there still be spaces available if I don't register my interest now?
MND-SMART is intended to be a long-term study. For the trial to be successful, we will need people with MND to join the trial in the coming months and years so that we can continue to test new drugs.

We will recruit people as quickly as we can however this is limited by the capacity of the teams in each trial centre to ensure participant safety and a high standard of patient care.

Can you pay travel expenses for participants?

Yes, we can pay some expenses for you to travel to your nearest trial centre for appointments. Additional grants may be available depending on where you live; please speak to your local trial centre for further information.

Does anyone stand to make money from the trial results?

No. The trial is led by the University of Edinburgh and run by academics and doctors in Universities and the NHS.

Will I get paid for being part of the trial?

No, you will not be paid for taking part. We can help towards travel expenses. Please speak to your local trial centre for information.

Trial Design

What drugs will you be testing first?

At the launch of MND-SMART, we will be testing two drugs against a placebo or dummy drug. Both of the drugs are already approved for use in other conditions. This is called drug repurposing. Participants will take either of the active drugs or the placebo.

The first drug is already used to improve the memory of people with Alzheimer's disease (a form of dementia) by reducing the action of a brain chemical called glutamate. It is thought that this drug may slow the damage to nerve/brain cells in people with MND.

The second drug is used in the treatment of anxiety and depression. It has been shown to protect brain cells in animal studies by slowing production of faulty proteins that can cause brain and nerve cells to die.

How did you select the drugs to be tested first?

To select the first two drugs for this trial, our scientists used an unbiased system to review all published research studies in MND and similar neurological disorders (e.g. Alzheimers and Multiple Sclerosis) diseases and produced a list of drugs that may slow, stop or reverse the progression of MND. We looked at studies in other neurodegenerative diseases alongside MND studies as the cells in the brain and body may be affected in a similar way in these diseases to MND.

Next, we produced a shortlist of drugs based on how well the studies were conducted, and how effective and safe the drugs were. A group of expert neurologists then reviewed the shortlisted drugs and decided which were most likely to slow, stop or reverse the progression of MND and be safe to take by people living with the disease. The two most promising drugs have been included in the trial.

How will you choose the next drug(s) to be tested?

MND-SMART is designed to run continuously for years to come. To help select future drugs we will trial, we will repeat the process of reviewing published information from MND and neurodegenerative disease studies at regular intervals.

We want to keep improving this drug~~s~~ selection process and so are developing new computer programmes to make compiling and reviewing published studies more efficient. We are also working on gathering further information on potential drugs by testing them in our labs on stem cells derived from people with MND.

Are you going to start stem cell trials?

We include all published studies on stem cells in our unbiased pipeline for drug choice. We are also working on gathering further information on potential drugs by testing them in our labs on stem cells derived from people with MND.

If, in the future, treatment with stem cells show the most promise for beneficial effects in MND, according to the scientific evidence, we may include them in MND-SMART.

If a drug is found to have a positive effect, how long will it take to become available to everyone?

We will work hard to make this happen as quickly as possible. If we find a drug leads to meaningful changes when we analyse the trial data, we will apply to the early access to medicines scheme. This is run by the Medicines and Healthcare Product Regulatory Agency (MHRA) who approve the use of all drugs in the UK. They will review the trial results and decide if, and when, the drug could be made available.

Please remember it will take many years to collect all of the data required to complete the final trial analysis required for this application.

How will you choose who takes which drug, and who takes dummy drug (placebo)?

People are assigned to a trial drug, or to the placebo, at random by a computer program. No-one involved in the trial can influence whether a participant receives a trial drug or placebo. This makes sure the results of the trial are not biased.

We need a placebo group so that we can prove any changes we see in the trial participants are due to the active drugs and not any other factors.