What are clinical trials?

A clinical trial is a scientifically controlled study exploring both the effectiveness and safety of a particular therapeutic treatment, such as a drug. Clinical trials are essential in identifying if any side effects of a new treatment are more threatening than the disease it is designed to treat.

There are different approaches to carrying out clinical trials, with the randomised clinical trial (RCT) typically considered as the 'gold standard'.

In an RCT, the impact of a treatment is determined by comparing groups of patients who do (treatment or experimental group) and do not receive the treatment (control or placebo group). The RCT approach means that participation in a clinical trial may involve allocation to the control or placebo group – with no treatment received.

Clinical trial stages

Any therapy approaching the first small-scale trials in humans will already have undergone a lengthy period of development and testing in the laboratory. The involvement of human patients only occurs at the last stage of testing before a drug is licensed for general use.

The standard model adopted when a new treatment is tested follows three consecutive stages: Phases I, II, and III.

Phase I

The first phase concerns establishing the safety of a potential new treatment, typically in a very small sample of people (5-20). Generally, this phase involves healthy volunteers rather than patients, with those taking part monitored for adverse reactions and side effects. New treatments will not advance beyond this first phase where any adverse reactions or side effects are judged to be too dangerous.

Phase II

The second phase establishes the optimal dosage, timing of doses, and the delivery route of the drug (i.e. orally, injection, etc.) for the next phase.

Phase III

The third phase aims to demonstrate that the treatment is beneficial for patients. In this stage, testing will normally involve hundreds of patients – this is enough to confirm that the treatment is effective. The results of this phase determine if a treatment is approved for use to treat diseases.

Trials for progressive disorders such as MND often last for over a year with each patient, involving long-term follow up to ensure that any effects of the treatment are of lasting value.

Participating in a clinical trial – what you need to know

In order for clinical trials to successfully establish if a treatment is effective, it is necessary that neither the people with MND nor the doctors conducting the clinical trial are aware of who is receiving the treatment and who is receiving the placebo. This is to ensure the omission of any bias in the way that patients or doctors may report the effects of treatment.

Participating in a clinical trial may involve no treatment being received.

Should you or someone you know wish to take part, it is important to firstly pay attention to the inclusion criteria which determine eligibility. Typical criteria include age, type and stage of disease, and previous or current treatments. Inclusion criteria ensures that clinical trials produce reliable findings.

All clinical trials have strict guidelines about who can and who cannot participate. It is commonplace for many willing trial participants to find themselves unable to take part. Though this may be disappointing, it is important to recognise that clinical trials are not treatments – they are experiments which aim to develop treatments.

As with participation in all research studies, taking part in a clinical trial will come with both benefits and risks, and it is important to consider both. Even after agreeing to take part, you will always be free to withdraw from the clinical at any time.

Some benefits to consider include:

- The opportunity to help others with MND by contributing to research
- The extra expert medical attention received throughout the trial
- The possibility that the trial treatment is in fact better than existing treatments, and is carried forward

Some risks to consider include:

- The time commitment to follow procedures, including travel
- The possibility that you may not be given treatment due to being allocated to a placebo group
- The potential for unpleasant side effects of the trial treatment

Participation will involve comparison of various outcome measures, such as the ALS functional rating scale. Use of such scales allows for comparisons on functioning over time such as on speech or handwriting.

Some broader questions to consider before deciding if you would like to get involved in a clinical trial include the following:

- How might involvement impact on my daily life?
- Who am I doing this for?
- Am I prepared to risk being on the placebo?