

Unproven treatments in MND

Information Sheet C

This information sheet presents the facts about alternative or 'unproven' treatments. It aims to provide information in order for you to make an informed choice about whether to go ahead with a treatment.

The content is split into the following sections:

- 1: **Conventional treatments**
- 2: **Complementary therapies**
- 3: **Alternative therapies**
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Disclaimer: Please note that information provided in this information sheet is based on a review of the currently available literature. This information sheet was written by MND Association staff who are not clinicians, so any information provided in this sheet should not be considered clinical advice. You should always discuss potential treatments with your clinician.



This symbol is used to highlight **our other publications**. To find out how to access these, see *Further information* at the end of this sheet.

1: Conventional treatments

A conventional treatment is one which has been approved for use as a treatment for motor neurone disease (MND), based on reliable scientific evidence assessing its safety and benefit for people with MND.

A treatment needs to be licensed and approved before it can be prescribed or recommended for use to treat an illness or condition. In the UK, the Medicines and Healthcare products Regulatory Agency (MHRA) licenses treatments. In Europe this happens via the European Medicines Agency (EMA).

Current treatments for MND available in the UK

At the present time, the only drug that has been proven to slow the progression of MND is riluzole. This treatment has been rigorously tested and there is reliable evidence that it is both safe and beneficial for people with MND.

In the absence of a conventional treatment that can cure MND or stop it from progressing, it is understandable that people living with MND may wish to explore other avenues.



For further information about riluzole, see: Information sheet 5A – Riluzole.

2: Complementary therapies

A complementary therapy is one that may make day-to-day life more comfortable for someone living with MND. However, while complementary therapy may help relieve symptoms such as anxiety or pain, the therapist will not claim that the therapy can cure or even slow the progression of the disease. Complementary therapies can be used alongside conventional treatment such as riluzole; examples include massage, aromatherapy, acupuncture and meditation.



For further information about complementary therapies, see: Information sheet 6B – Complementary therapies.

3: Alternative treatments

Alternative treatments, also known as unproven treatments, may appear to offer some hope and an opportunity to take a proactive approach to tackling the disease.

Drugs which are not licensed to treat MND fall into the 'alternative treatments' category.

Stem cell therapies and supplements or diets specifically claiming to cure or treat MND would also fall into this category. The people or organisations offering these treatments may suggest that they will work better than conventional treatments. However, these remarkable claims are usually not supported by any reliable scientific evidence.

How do you prove a treatment works?

All potential treatments for MND need to go through clinical trials, to test their safety and effectiveness. Those offering unproven treatments often refer to anecdotal evidence and personal testimonies to support their claims a treatment works. However, clinical trials take measurements of disease progression such as ALS Functional Rating Scale (ALSFRS-R) scores or measures assessing respiratory function or muscle strength to provide evidence about a treatment's safety and effectiveness.

Should I take unlicensed or unproven treatments outside of a clinical trial setting?

Some MND treatments currently being tested as part of clinical trials might be available to purchase on the internet or be offered by clinics. Caution should be taken when considering treatments that have not been approved or licensed for MND. Stem cells are an example of a treatment still undergoing testing though clinical trials. These trials are establishing if stem cells are a safe and effective treatment for MND.



For further information about stem cell therapy and research, see: Information sheet F – Stem cells and MND.

Are there risks in taking treatments being used for, or undergoing trials in other illnesses and neurodegenerative conditions?

Even if a drug or treatment has shown no serious side effects in humans as part of clinical trials for other illnesses, it still needs to undergo clinical trials in MND before it can be licensed specifically for treating this disease. This is to establish:

- whether there are any serious side effects associated with the drug,
- if the drug interacts with other medications (such as riluzole),
- if the drug shows a beneficial effect what the correct dose should be.

4: Proving a treatment through clinical trials

The gold standard process to establish both the safety and effectiveness of a new treatment is a series of tests in humans known as clinical trials. A treatment without published clinical trial results:

- lacks reliable evidence to support its effectiveness,
- may have unacceptably serious side effects,
- has not been independently examined,
- could be without scientific rationale.

To get as far as starting the clinical trials process, a new treatment must have sound scientific reasoning behind it and show promise in laboratory tests. Once the treatment makes it to the clinical trials stage, researchers will carefully monitor a large number of people with MND who are receiving the trial treatment and compare their progress with that of a similar-sized group of people with MND who are receiving a placebo, or 'dummy' treatment.

Participation in any trial, however rigorously conducted, involves an element of risk. There are various ways in which researchers ensure that the results of clinical trials are reliable:

- By involving large numbers of people in the trial, researchers can account for the fact that there are variations in the way that MND affects individuals, reducing the possibility that any apparent effects of the trial drug are just down to chance.
- The placebo group allows researchers to get a true picture of the effects of the trial treatment and ensures that any apparent beneficial effects are not due to the power of positive thinking, the extra attention from medical staff that comes with participating in a trial, or any other factor.
- Double blinding: neither the people with MND nor the doctors carrying out the clinical trial will know who is receiving the placebo and who is receiving the trial treatment. This eliminates the possibility of bias in the way that the participants or doctors report the effects of the treatment.

In addition to establishing whether a treatment is effective, clinical trials answer other important questions:

- Is the treatment safe and what are the risks associated with taking it? For example, does the treatment make the disease worse, or are the side effects of the treatment worse than the disease itself?
- What is the best dose for a new drug? How is the drug processed by the body? Once a clinical trial has been completed, the methods and results will be scrutinised by others in the scientific community before being published in medical journals.

People with MND who take part in properly conducted clinical trials are never expected to pay for the experimental treatment.



For further information about clinical trials, see: Information sheet D – *Clinical trials*.

5: What about testimonials from people with MND as evidence?

Publications that promote unproven treatments often include testimonials from a handful of people for whom the treatment has apparently been very successful. Such stories may also occasionally appear in the press or on the internet. When considering these testimonials, it is important to remember that:

- A person's belief that a treatment will help them can bring about apparent beneficial effects. This 'placebo effect' is a normal response and can produce some improvement immediately after treatment. However, this is short-lived, perhaps lasting a few weeks, and is followed by deterioration.
- People sometimes feel that they should feel better after they have tried a new treatment, particularly if they have invested a great deal of effort and/or money in getting that treatment, or if others have contributed towards the cost. This can affect the way they report how they feel.
- Everyone's experience with MND is different. The disease naturally progresses more slowly in some people than in others. The fact that one or two people appear to live longer or have slower disease progression cannot be attributed to the treatment alone.
- The people reporting the beneficial effects of the treatment may not actually have MND. There are various diseases besides MND that affect motor neurones and cause symptoms very similar to those seen in MND. However, these diseases have different underlying mechanisms and may progress in a totally different way to MND.
- People react differently to treatments/drugs, and effects of the treatment may not necessarily be the same for each person who takes it.
- People may have been taking several unproven treatments at a time. This makes it hard to tell which may have been causing the observed change or beneficial effect (if any). In clinical trials, the other drugs or treatments a participant can take while taking the study drug/treatment is usually monitored.

6: Finding the facts about unproven treatments

Sense about Science

Sense about Science is an independent charity that challenges the misinterpretation of science and evidence in public life. From news stories to online testimonies, they encourage the public to challenge scientific and medical claims and ask 'does the evidence support the conclusions being made?'

The MND Association worked with Sense about Science to help produce their 'I've got nothing to lose by trying it' information booklet so that people living with MND can make up their own minds about unproven treatments.

For further information on Sense about Science, and to download the information booklet, see the Sense about Science resource (find the link in the 'How do I find out more?' section).

ALS Untangled

ALSUntangled was started in 2009 by a group of neurologists and researchers to scientifically review off-label and unproven treatments, to allow people with MND to make more informed decisions about these treatments.

These researchers give their opinions and first-hand knowledge of unproven treatments to look at exactly what is on offer, the cost, the science behind the claims, and the risks and benefits.

Once enough information has been collected ALSUntangled publicly release a summary in the journal *Amyotrophic Lateral Sclerosis and Frontotemporal Degeneration* so that people with MND and their families can make their own decisions regarding that particular treatment. If you want to pursue one of the off-label or unproven treatments that they have reviewed, you can make up your mind based on the most important factor to you. This could be if there is evidence of side effects, or if there is any scientific basis for the treatment working.

If you hear of an off-label or unproven treatment, and ALSUntangled have not previously investigated it, you can submit the suggestion to them via their 'Twitter' account (@ALSUntangled) or by voting for it on their website www.alsuntangled.com.

For further information on ALSUntangled, and to view their current lists of investigations and completed reports, see the ALSUntangled resource (find the link in the 'How do I find out more?' section).

7: Questions to ask about unproven treatments

When deciding whether to undertake an unproven treatment, it may be helpful to consider the following:

What does your own doctor think?

It is always advisable to discuss any treatments you are considering with your neurologist or GP. Some treatments may interfere with other medications and your doctor will be able to provide you with expert medical advice. Beware of those offering unproven treatments who discourage you from talking to your doctor.

What evidence is there to support the claims made about the treatment?

Published results from clinical trials are the only reliable evidence of the effects of a treatment – you should never be expected to pay for a treatment that lacks this evidence. The Research Development team at the MND Association or your neurologist will be able to tell you if the results of research on a particular treatment have been published in medical journals. If the clinic tells you that their treatment is 'clinically proven', ask for the name of the journal in which the results have been published. A report on a web page is not acceptable.

What are the risks involved?

The possible side effects associated with a treatment should always be clearly stated, so that people can make an informed choice about whether to go ahead. Ask how the clinic follows its patients after the treatment and how side effects have been assessed and documented. Even those treatments described as 'all natural' are not necessarily safe.

Other important risks to consider are:

- If the treatment involves a long and tiring journey to reach the clinic, how will this affect your health?
- How will you and your family feel if the treatment fails?
- Is paying for the treatment going to put you and your family in a difficult position financially?

Where did you find out about the treatment?

Newspapers, magazines, social media sites and websites may have little regard for the presence or absence of good scientific evidence. The internet is also very difficult to police and so can be used to advertise and sell unproven treatments with relative ease. Safe and effective treatments will be endorsed by your doctor and the MND Association.

Do you have to travel to another country to receive the treatment? If so, why is it not available in the UK?

UK regulations surrounding medical practice and treatment exist to protect patients. Many countries have more lax regulations and policing, providing opportunities for those wishing to profit from the sale of unproven treatments. Bear in mind that some organizations offering unproven treatments (particularly stem cell treatments) will have

a contact address or telephone number in a country like the USA, via which enquiries and arrangements are made. However, the clinic itself will be in a less well-regulated country – ask where you will actually receive the treatment.

Are there claims that the treatment is effective for a wide variety of diseases?

Even when their symptoms are similar, different diseases involve different disease mechanisms and need to be tackled in different ways. It is unlikely that one treatment could be effective for a whole list of conditions. Most genuine treatments are of benefit in just one or two diseases.

8: MND Association policy statement on unproven treatments

There is currently no cure for motor neurone disease, which is why the MND Association is committed to funding and promoting scientific research into possible causes and treatments.

Only a drug called riluzole has been proved through clinical trials to be a successful treatment for people with MND. It extends life by up to three months.

Even so, there are many other unproven treatments marketed throughout the world for people with MND. However, their effectiveness has not been proved through clinical trials, and the science behind them is sometimes not well understood. The Association does not endorse these treatments.

In addition, we believe that allowing treatments to be made available which have not been through the established clinical trials process encourages unscrupulous individuals to market ineffective treatments for monetary gain.

We would encourage anyone with MND who is considering embarking on an unproven treatment to discuss all the implications with their neurologist before making a decision.

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9: How do I find out more?

Useful resources

We do not necessarily endorse any of the following organisations but have included them to help you begin your search for further information.

The contact details are correct at the time of publishing but may change between revisions. If you need help to find an organisation, contact the Research Development Team (see *Further information* at the end of this sheet for details).

ALS Untangled

International group of researchers that give their opinions and first-hand knowledge to look at the science, risks and benefits behind unproven treatments. To view the completed reports, or to submit a suggestion, please visit their website for details.

Website: www.alsuntangled.com

Sense about Science

A charitable trust that equips people to make sense of science and evidence. We worked with Sense about Science to produce the information booklet 'I've got nothing to lose by trying it' on unproven treatments. You can request a copy using the information below.

Address: 2 Stephen Street, London, W1T 1AN

Email: hello@senseaboutscience.org

Website: www.senseaboutscience.org

Tel: +44 (0)20 7490 9590

Download the booklet: bit.ly/SaS-booklet

Further information

You may find these information sheets from the MND Association helpful:

6B – *Complementary therapies*

5A – *Riluzole*

D – *Clinical trials*

F – *Stem cells and MND*

We also provide the following guides:

Living with motor neurone disease – our main guide to help you manage the impact of the disease

Caring and MND: support for you – comprehensive information for unpaid or family carers, who support someone living with MND

Caring and MND: quick guide – the summary version of our information for carers

You can download most of our publications from our website at www.mndassociation.org/publications or order in print from the MND Connect helpline, who can provide further information and support.

MND Connect can also help locate external services and providers, and introduce you to our available services, including your local branch, group, Association visitor or service development manager.



MND Connect

Telephone: 0808 802 6262

Email: mndconnect@mndassociation.org

Research Development Team

Telephone: 01604 611 880

Email: research@mndassociation.org

MND Association website and online forum

Website: www.mndassociation.org

Online forum: forum.mndassociation.org or through the website

We welcome your views

Your feedback is really important to us, as it helps improve our information for the benefit of people living with MND and those who care for them. If you would like to provide feedback on any of our information sheets, you can access an online form at: www.surveymonkey.co.uk/r/infosheets_research

You can request a paper version of the form or provide direct feedback by email: research@mndassociation.org.