

Secondly, it is very important that you agree with your DM1 doctor/nurse to come to all the monitoring appointments that are needed. If you do not come to clinics, your GP will stop prescribing Modafinil.

Then, if you wish to try Modafinil, your GP will be asked whether they are willing to prescribe Modafinil. This step is important because Modafinil is not licensed for DM1 sleepiness and needs to be prescribed “off label”.

Where can I get more information?

You can read the EMA ruling in full at http://www.ema.europa.eu/ema/index.jsp?curl=pages/medicines/human/referrals/Modafinil/human_referral_000236.jsp&mid=WC0b01ac05805c516f

*The UK survey is called “Modafinil for excessive daytime sleepiness in myotonic dystrophy type 1 – The patients’ perspective”. The authors were D. Hilton-Jones, [†]M. Bowler, H. Lochmueller, C. Longman, R. Petty, M. Roberts, M. Rogers, C. Turner, D. Wilcox. It is published in a medical journal called Neuromuscular Disorders (issue 22, pages 597–603, 2012). Your DM1 doctor/nurse can give you a copy if you would like.

You may find it helpful to talk to friends, family or your GP when you are considering a trial of Modafinil.

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Scottish Muscle Network

A TRIAL OF MODAFINIL:

WHAT YOU NEED TO KNOW

This leaflet is for people with Myotonic Dystrophy (DM1) to help them decide whether to have a trial of Modafinil.

Scottish Muscle Network can be contacted via email nss.smn@nhs.scot

Why have I been offered Modafinil?

Modafinil is a medicine that some people with DM1 find helps their excessive sleepiness. Others do not find it helpful. Your DM1 doctor/nurse has given you this leaflet so you can decide whether you would like to try it, to see if it works for you.

How does Modafinil work?

Modafinil acts on the brain to make people feel more awake. We know that it stimulates several chemical pathways in the brain. However, the exact detail on how it works is unclear.

Is Modafinil safe?

There are very rare side effects of Modafinil that can be serious and even life-threatening. These are:

- Serious psychiatric disorders (suicidal thoughts, mania and symptoms of psychosis such as delusion).
- Skin reactions, including severe reactions such as Stevens-Johnson syndrome, a life-threatening type of allergic reaction affecting the skin and mucous membranes. This risk seems highest in children.
- High blood pressure and irregular heart beat.

The European Medicines Agency (EMA) reviewed the research evidence on effectiveness of Modafinil in 2010. They advised that the use of Modafinil should be restricted to people with one rare condition, narcolepsy. This is because there was only good research evidence showing Modafinil helps symptoms in narcolepsy. The EMA advised Modafinil should not be used in other conditions.

The Medicines and Healthcare products Regulatory Agency (MHRA) are the UK agency who regulate which medicines can be safely and effectively prescribed. They balance the research evidence on whether the medicine works, against its side effects, and if they feel the benefits outweigh the risks then they issue a licence for the medicine. In the UK, Modafinil is only licenced for narcolepsy. It is not licenced for DM1 sleepiness.

Why would I try a medicine that has serious side effects?

The safety agencies above, and all doctors, aim to balance a medicine's benefits against the risks. There are no large, long-term trials of Modafinil in DM1, so the agencies cannot make that assessment. However, in small research trials, Modafinil improved sleepiness in some people with DM1. These trials only lasted a few months. A UK survey* of people with DM1 who had taken Modafinil long term showed that some people found it helpful but others did not. Many expert DM1 clinics in the UK and

abroad still prescribe Modafinil because the doctors feel it works for some people. The DM1 clinic doctors and nurses in Scotland work together as part of the Myotonic Dystrophy subgroup of the Scottish Muscle Network. This group agrees that Modafinil might be helpful for carefully selected people with DM1 sleepiness, where it is seriously impacting upon their everyday life.

This means your GP has to decide whether to prescribe Modafinil to you without a licence - this is called an "off label" prescription.

Are there other side effects?

About one in five people get headaches when they start Modafinil but this usually wears off after a few days.

Like all medicines, there are a range of side effects seen in some people. They will be listed on the information leaflet in the medicine packet and you should read that leaflet carefully.

What happens in a trial of modafinil?

First, some basic health checks are needed to be sure Modafinil is suitable for you. You need to have an ECG heart tracing, an echocardiogram heart scan and a breathing assessment. If you have had these recently, the results will be checked.