

CLINICAL TRIALS in MDS

Information for patients and family members

You may be asked to take part in a clinical trial by your physician as part of your treatment.

Or – you may want to ask your physician or specialist nurse whether a particular trial might be available – as part of your on-going care for MDS.

Patients need to be aware of clinical trials and need to be well informed by their physician or clinical nurse about the various points to consider before agreeing to taking part in a clinical trial: for example - **risks and benefits, regular travelling to the trial hospital, travel costs, understanding the consent forms, care after the trial ends**, etc.

It is important to know all these things and have answers to all of your questions before you become involved in a clinical trial.

What is a clinical trial ?

Clinical trials are the process by which new treatments are tested, evaluated, and the evidence gathered so that the decisions can be made about changing standard practice.

Trials aim to find out if a new treatment or procedure is safe, has side effects, works better than the currently used treatment, helps you feel better or might be a cure for a condition.

There are four phases of clinical trials. Each treatment being tested has to go through all these phases before it can be used. Patients are usually involved in phase 3 and 4 trials.

Phase 1 trials are often 'first-in-man' (treatments never been tried on human patients before). Drug trials will be looking for safety information, side effects, dosage issues. Phase 1 trials will include a maximum of 10-15 patients. Phase 1 trials are done under rigorously controlled conditions with intensive monitoring.

Phase 2 trials examine which diseases respond to the new treatment, and will be comparing dosages, assessing side effects and looking at outcomes. The numbers of patients recruited may be quite small (50 or so) but the data gathered will be the basis for later larger scale trials.

Phase 3 trials are most often 'randomised controlled trials' (or RCTs). This may involve comparison of a new treatment with a placebo or with a standard therapy. If it gives better results, it may become the new standard treatment. Patients are randomly selected to receive one of the treatments. These trials are mostly large scale and can have thousands of patients in them, although with a rare condition like Myelodysplastic Syndromes the numbers are more likely to be in the hundreds.

Phase 4 trials are carried out after a drug has been licensed – they collect information about side effects, safety and the long term risks and benefits of a drug by continuing surveillance of patients on the treatment. This may be how rare side effects are identified.

Different types of trials

The researchers may look at the impact a treatment has on you personally as well as the treatment's clinical benefits – for example, how often you have to travel to the hospital, or whether you are able to lead a full and normal life. Studies of impact and side effects are sometimes called quality of life studies. Most well planned trials include a quality of life study.

Usually, a new treatment has to go through a few phase 3 clinical trials before doctors are confident enough to accept it as the new standard treatment. One good trial result could happen by chance or because of the design of the trial. This is not likely if several trials have the same results. Satisfactory results in a number of clinical trials are essential before a new treatment can be recommended by a regulatory body such as NICE.

Not all clinical trials will result in new and better treatment. Some will discover that the treatment being tested does not work, or is no better than an existing, established treatment. A trial might find that a new treatment has side effects that are worse, or no less, than with existing treatments. But this information is also useful for researchers and doctors. and in the end for patients.

Other aspects to consider

As patients and patient support groups, we also have the ability to shape the future development of clinical trials:

- We must request the publication of all clinical trials (currently only 40% of trial results are published).
- We have to request that patients who participate in clinical trials and benefit from the drugs be given that treatment for as long as they need it – not just for the time of the trial.

Please contact us on 0207 733 7558 if you have any general questions about clinical trials.

Please always consult your doctor or nurse about any decisions regarding your treatment.

Notification of a current clinical trial in Phase 3: *TELESTO* trial

Researchers at selected NHS hospitals are offering MDS patients the opportunity to take part in the **Clinical Research Study called *TELESTO*** for the treatment of **Iron Overload** due to regular blood transfusions.

Who can participate in the study?

Adult patients with low or intermediate-1 risk MDS who have too much iron in their blood (Serum ferritin level above 1000mg/L) due to a lot of blood transfusions (received at least 15 units of blood).

Eligible participants will undergo medical examination and assessments at no cost, and may also be compensated for their time and travel.

For more Information contact:

Lead Co-ordinating Research Centre
Dr Paresh Vyas, Churchill Hospital,
Oxford.
Telephone: 01865 572023

Or visit the Patient Information websites:

www.public.ukcrn.org.uk/search

www.cancerhelp.org.uk