

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 trial might be available at your local hospital or another treatment centre, as part of your on-going or future care for MDS. Good trials are crucial in MDS and very worthwhile discussing at any point.

Patients need to be aware of clinical trials generally and be well informed by the clinical team about the various points to consider before accepting or declining to take part. For example - **risks and benefits, travel requirements to the trial hospital, travel costs, understanding the consent forms, monitoring tests required, care after the trial ends**, etc.

It is important to take your time and have answers to all of your questions before making your decision. Equally, it is important not to be afraid of trials nor to dismiss a trial option too quickly, as it can offer benefits. Talking to support groups and other patients can also help.

What is a clinical trial ?

Clinical trials are the process by which new treatments are tested and evaluated in order to gather the evidence necessary for making decisions 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 MDS 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 its 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. All well planned trials should include a quality of life assessment.

Usually, a new treatment has to go through a few phase 3 clinical trials before doctors are confident enough to accept it as a new standard option. One good trial result could happen by chance or because of the design of the trial. This is not likely if several trials come to the same conclusion. 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 support groups, we 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 benefit from the treatments received in clinical trials are given that treatment for as long as they need it – not just for the duration of the trial
- **Your participation in a clinical trial will not affect your care and any treatment received in a trial will be given in addition to other standard 'best-proven' treatments available**

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

Always consult your doctor or nurse about any decisions regarding your treatment

Brief outline of some interesting current clinical trials in MDS:

Many more trials available – consult websites below and your clinical team

ELASTIC: for MDS Intermediate 2 or high-risk, CMML or AML patients who also have low platelets. For this study patients are given azacitidine in combination with a drug called eltrombopag - which can boost the production of platelets. Patients who have already had azacitidine or other platelet boosting drugs are not eligible.

AZA003: for low-risk MDS patients who need blood transfusions at least every 4 weeks and have low platelets. In this Phase 3 trial, the oral (tablet) version of the MDS drug azacitidine is tested, to compare its efficacy with the currently widely used sub-cutaneous (injectable) version.

De-Iron: for MDS patients who are just starting to receive blood transfusions. This study tests whether giving the iron chelation drug deferasirox at an earlier stage is better than using it when iron levels start to rise.

**For full details, recruitment criteria,
explanations and locations of these trials:**

Call or email MDS UK

Ask your haematologist or nurse specialist

Or visit the following websites:

www.mdspatientsupport.org.uk

www.cancerhelp.org.uk

www.clinicaltrials.gov