MDS UK
Supporting Patients with Bone Marrow Failure
www.mdspatientsupport.org.uk

MDS Factsheet

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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

Clinical trials in 2019/2020 – brief selection and outline:

There are a number of trials open, or due to open for a variety of patients:

- Patients with MDS that no longer responds to hypomethylating agents (eg. Azacitidine) or for whom hypomethylating agents never worked: MEDI4736 is a Phase 1 trial running in KCH, London and Brighton.
- Very specific sub-groups of MDS, with an IDH genetic mutations: FT-2102, a Phase1/2 trial available at University College London Hospitals and Royal Marsden Hospital, Sutton. AG-221-AML005, a Phase 1/2 trial for those unable to have intensive induction chemotherapy.
- High-risk patients who are unable to undergo a stem cell transplant: RFUSIN2AML2, a Phase I study of Immune Gene Therapy.
- Trials that combine existing drugs with a new agent, with a view to improving efficacy: Panther, a Phase 3 trial for high risk MDS, CMML or low blast count AML. Available in Bournemouth, Swansea, Bart's and Kent. BRIGHT, a Phase 1B trial available in Oxford.
- Transfusion dependent patients: FibroGEN and Luspatercept are due to open soon.

For the complete list of trials and all details, call us, or look up "Current Clinical Trials" on our MDS UK website. Patients and carers are encouraged to check that page regularly and ask about trial options at their local hospital, as well as at hospitals further afield.

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