Blood and Lymphatic cancers
Leukaemia CARE
supporting a quality of life

Advocacy Toolkit:

Clinical trials – the basics

What is the point of clinical trials?

Clinical trials of new treatments occur in different stages and are designed to assess the safety/side effects, necessary dosage, effectiveness in treating the disease and the long-term results.

Phase 1 – This is the first human trial and often includes only a small group of healthy volunteers or patients who have run out of treatment options. The primary goal at this stage is to test the safety of the treatment by steadily increasing dosage; this will only be done if there are minor or no side effects.

Phase 2 – A larger trial involving patients with the disease the treatment is targeting. The main aim of this phase of the trial is to assess the effectiveness against fighting the disease. A group of patients involved at this stage may receive a placebo treatment, read below to find out what this involves in cancer trials.

Phase 3 – The biggest trial of the treatment before approval, involving more patients over a longer period of time. This trial is to assess long term effectiveness and safety of the treatment. During this phase the treatment will be compared to others that are already available, in order to assess whether it is beneficial in practice.

Phase 4 – This may not occur for all treatments, but the phase involves long-term assessment and review of the treatment after it has been approved for use.

Placebos – what are they and could I be given one?

Placebos look very much like the real treatment, except they do not contain any active form of medication. The placebo group are a control group used to determine whether the new treatment is causing improved outcomes.

Not all cancer treatment trials will have a placebo group. In these trials, patients will either receive the trial treatment or the standard of care (SOC), the best available treatment already approved for use. The outcomes of patients will then be compared to assess the impact the trial treatment is having.

If a placebo is used in a cancer trial, patients will be treated with the SOC in addition to either the trial treatment or the placebo. This ensures that patients are receiving the best available treatment whilst still allowing fair comparison of outcomes between patients given the placebo and those given the trial treatment.

However, you are unlikely to know if you are given the placebo treatment (blind trial) and, in many clinical trials, the people giving you the treatment will not know either (double-blind trial). This is to avoid bias results and the 'placebo effect'.

Fun fact

The 'placebo effect' – an improvement of a patient's disease when given placebo drugs. This is due to psychological effects of the patient either thinking the drug will make them better or because they feel better cared for during the trial.

Are there any risks of being involved in a clinical trial?

The effect that a new treatment has on the human body cannot be guaranteed prior to human clinical trials. There is a risk, therefore, that treatment may be not effective in fighting the disease, leading to deteriorating condition, or could have unexpected side-effects.

All new treatments undergo thorough testing before human trials begin and are reviewed by a number of regulatory bodies before authorisation is given . This ensures that there is significant evidence to suggest that the treatment will be beneficial and that participants will not be put at undue risk of harm.

What are the benefits of being involved in a clinical trial?

Increased level of care

Clinical trials have rigorous procedures that mean participants will be required to visit their doctor more often and undergo extra tests or monitoring that they would not normally have. This ultimately can help patients to feel more cared for.

You must consider, however, that this would require extra time commitments, but you are able to withdraw from a trial if your circumstances change and you can no longer commit the time.

Access to new drugs that aren't normally available

While you do have to consider the potential negative effects of trial treatments, taking part in a clinical trial means that you have access to treatments that aren't available to the general public and could be very effective in fighting your disease.

For example: Imatinib trials for CML patients saw exceptional results , with one study finding that after 5 years of treatment 98% of patients had normal white blood cell count and survival rate was 89%. Imatinib is now the main first line treatment for CML.

Here is an article from 2008:
http://www.nature.com/scitable/topicpage/Gleevec-the-Breakthrough-in-Cancer-Treatment-565

You are helping to improve treatments for other people

Regardless of the outcomes of the trial, ultimately your participation helps researchers to find out more about how a treatment works within the human body and whether it is effective and safe at fighting a disease. Ultimately, this shapes not only that specific treatment but the development of further treatments also. Essentially, your involvement helps to make medicine better for future patients.

Who regulates clinical trials?

Clinical trials are tightly regulated to ensure safe and ethical practice.

The Medicines and Healthcare Products Regulatory Agency (MHRA)
A government body that review and authorise clinical trials – including reviewing the healthcare site whereby trials will be run and regulating medicines used in the UK.

Scientific review
A group of independent researchers (leading experts in the field, who are not associated with the trial) review the science behind the treatment and the protocols to be used during the trial phases.
Health Research Authority (HRA)

An NHS group that are responsible for the ethics committees across the country, whether private or NHS funded.

Data monitoring committee
Responsible for analysing the data being taken from the clinical trials and will stop a trial early in the case of treatments being very effective at treating a condition compared to a previously established treatment, as this means that patients not receiving the new treatment are being treated unethically. Alternatively, a trial will be stopped if the treatment is posing too many risks for participants.

What are my rights during a clinical trial?

Patients must have given informed consent when taking part in a clinical trial. This means:

- Participants must be given full information about the purpose of the trial, the procedures (both with treatment delivery and running of the trial), and what positive or negative effects it is expected the treatment may have.
- Participants are given the opportunity to meet with the researchers and ask questions during or after this time.
- Participants give written consent accepting to be involved in the trial AFTER receiving all the information about the trial.
- Participants can refuse to participate at any stage of the trial.

Where can I find out more?

The UK Clinical Research Collaboration has produced a booklet on understanding clinical trials:

http://www.ukcrc.org/wp-content/uploads/2014/03/iCT_Booklet.pdf

NHS Choices – a good basis with patient stories and further links to find out more:

http://www.nhs.uk/Conditions/Clinical-trials/Pages/Introduction.aspx

How can I get involved in clinical trials?

In some cases, your healthcare professionals may be involved a clinical trial that is happening and may invite you to take part. They should have been fully briefed on the trial and be able to answer any questions you may have, helping you to make an informed decision on taking part.

This may not always be the case, however, so you may wish to find a clinical trial yourself. Just ensure, when doing so, that you use trusted sources for finding clinical trials, for example:

NHS: http://www.nhs.uk/Conditions/Clinical-trials/Pages/clinical-trial.aspx

Cancer Research UK: http://www.cancerresearchuk.org/about-cancer/find-a-clinical-trial

UK clinical trials gateway; https://www.ukctg.nihr.ac.uk/clinical-trials/search-for-a-clinical-trial/

Global trials: https://clinicaltrials.gov/

Further questions

If you have any further questions about clinical trials then you can contact our Campaigns and Advocacy team. They are available Monday to Friday from 9:00am – 5:30pm. If you would like to speak to them, you can:

- Call our office line on 01905 755977
- Send them an email at advocacy@leukaemiacare.org.uk
- You can also call the 24-hour CARE Line, free of charge on 08088 010 444. The team will pass your enquiry onto the Campaigns and Advocacy team.

Please note that our Campaigns and Advocacy team are unable to provide:

- Detailed medical advice or recommendations
- Legal advice
- Advocacy for a course of action which is contrary to the aims and objectives of Leukaemia CARE