



Children's
Cancer and
Leukaemia
Group

Taking part in clinical trials

Information for parents and carers of a child or young person with cancer



www.cclg.org.uk



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Edited by the CCLG Publications Committee, comprising multiprofessional experts in the field of children's cancer. The original version of this booklet was written in conjunction with the CCLG Patient Advocacy Committee.

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Many children and young people with cancer are treated on clinical trials. We hope this booklet, designed for parents and carers of children and young people with cancer, will help you to understand more about clinical trials and answer some of the questions you may have.

Always discuss any questions or specific queries relating to treatment or participation in a trial with your child's doctor or other members of the team.

What is a clinical trial?

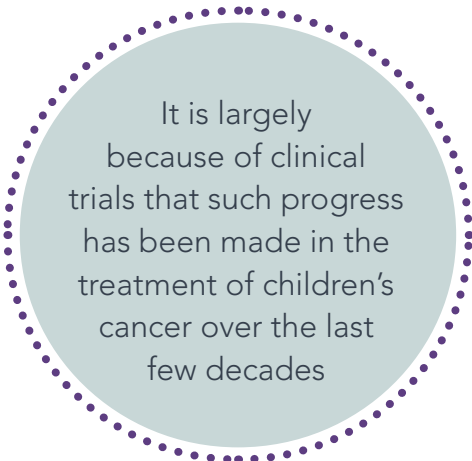
A clinical trial is a medical research study involving people to find out the most effective treatment for a particular disease. Before a new treatment is available to all patients, it must be tested to be sure it is safe and effective. This is done through clinical trials.

The aims of clinical trials are to:

- find out whether a new treatment or procedure is safe
- find out whether a new treatment or procedure has any side effects
- test new medicines
- see whether a new treatment or procedure works better than the currently used treatment
- find out which treatments have the least impact on patients' everyday lives
- find out which supportive care treatments can help reduce side effects
- what the impact of the cancer and the treatment has on quality of life

Why are clinical trials important?

As we don't yet know the best way to treat every type of cancer, clinical trials help us to find better ways of treating the different kinds of cancer. Clinical trials allow us to test new treatments and ways of controlling symptoms, or to investigate new ways of preventing or diagnosing cancer.



It is largely because of clinical trials that such progress has been made in the treatment of children's cancer over the last few decades

Are there different kinds of trial?

Yes. There are three different kinds (known as phases) of trial. Each phase aims to find out something different about the new treatment or procedure.

Phase I trials

test new treatments on people for the first time. They help us find the correct doses of new drugs and any possible side effects. These trials are carried out in a small number of patients, who have had all available standard treatment. Usually a new drug has been tested in adults before it is offered to children.



Phase II trials

test to see whether a treatment is likely to be effective at the dose(s) chosen in phase I. They aim to find out how well the new treatment works for particular types of cancer and to highlight any unwanted side effects. They will again involve a relatively small number of patients.

Phase III trials

compare the new treatment to the best existing treatment, or 'standard treatment'. These trials involve larger numbers of patients and usually run for much longer than phase I and II trials.



All these types of trials may run across different countries at the same time



Can anybody enter a trial?

There are very clear guidelines about which patients are eligible for a particular trial. Each trial will have specific rules about who can take part, such as the type of cancer, age of patient and stage of the disease. Your child's doctor will explain this to you.

What are we told about the trial?

Detailed information sheets are provided for patients and parents, and there will be opportunities to discuss the trial with the doctor or nurses, and to ask any questions. The information sheets will give you details about the treatment and any possible side effects, as well as explaining what will happen to the data collected during the trial.

A research nurse, or another member of the team, will be available to explain the trial in more detail, answer any questions you have, and talk through anything that is not clear from the information sheets.

Who is responsible for running the trial?

Clinical trials might be developed by hospital teams, research scientists or by pharmaceutical companies. The trials may be national or international. Your child's research team can include nurses, pharmacists, clinical trial practitioners and data managers who work across a huge variety of research projects.

Clinical trials need approval from a Research Ethics Committee and the Health Research Authority to make sure the trial is safe and ethical.

What happens if we decide to take part?

Once you have read the information sheets and had a chance to ask any questions, you will be given some time to think about whether or not you wish to go ahead. The length of time will vary according to the trial, but will usually be at least 24 hours.

In order to take part in the trial, it will be necessary to sign a consent form to confirm that you understand what happens in the trial and you agree for your child to take part. This will be signed by you as the parents or the patient themselves, depending on age.

What happens if we don't want to take part?

The doctor treating your child will respect your choice and your child will receive the best currently known and proven treatment.

What if we say 'yes', then change our minds?

Patients and parents can change their mind at any time. You do not have to give a reason if you do not wish to. Your child's doctor will respect your decision and your child will then receive the best known and proven treatment.



Does everyone in a trial get the same treatment?

Once you have given consent for your child to take part, your child's doctor or research nurse will register them on the trial. If a trial is randomised, a computer will assign your child the treatment 'arm' they will be given - either the new treatment or the best treatment that is currently available. This makes sure there is an even split of patients across the trial so an accurate comparison can be made. It also means each patient has a fair and equal chance of being allocated either treatment. Sometimes a trial may contain more than one randomisation. Your child's doctor will explain in more detail about how randomisation works, and precisely what it means in any particular trial.

Is the treatment safe?

All the possible risks and benefits of taking part will be explained to you. Once the trial has started, it is then reviewed on an ongoing basis. If there are any concerns about the safety or effectiveness of the treatment, the trial may be stopped, and treatment will continue using the current best known treatment. It is very rare that this would happen.



The safety of patients in clinical trials is of the utmost importance. All trial protocols have to be reviewed and approved by ethics and regulatory committees.

Clinical trials are very closely monitored by a number of different individuals and organisations. This will include the Chief Investigator (overall lead clinician), the working group which has developed the trial, and relevant staff within the clinical trials unit.

An Independent Data Monitoring Committee may also be established to oversee the conduct of the trial. If there are any concerns about the conduct of the trial or the results, a trial may be stopped early.

What are the benefits of taking part in a clinical trial?

- your child may receive a new treatment that is only available in a clinical trial
- your child's treatment will be the same, wherever you live
- national, or often international, experts in the particular tumour type will have worked together to develop the trial protocol
- there is considerable emphasis on patient safety and your child will be monitored closely
- sometimes there may be no benefit for your child but the results of the trial may help doctors improve cancer treatments for future patients

Are there any disadvantages?

Your child's doctor will discuss with you any possible disadvantages. Depending on the design of the trial protocol:

- you may have to make more hospital visits
- you may have to travel further to a specific hospital if the trial is not open at your local Principal Treatment Centre
- your child may have more tests carried out
- the new treatment, although expected to be better, may not actually be better
- your child may experience side effects that you or your doctor are not expecting, but you will be closely monitored
- your child may not be able to have the drug treatment made up specially as a syrup, but will have to swallow tablets/capsules the same as other patients on the trial.

How many patients are needed?

The trial protocol contains details of how many patients are needed. Statisticians advise on the number of patients needed in order to effectively answer the question(s) posed within the trial. The numbers will vary depending on the type of trial. For many trials of childhood cancer, patients will be recruited from treatment centres in the UK and Ireland, as well as from countries overseas. This helps to ensure recruitment is completed as quickly as possible.

Where does treatment take place?

The trial treatment will take place in a hospital with doctors who are specialists in children and young people with cancer (paediatric oncologists). For most clinical trials, this will be the same hospital where patients are treated with the best known standard treatment. However, for some trials your child's doctor may refer you to another specialist hospital where the trial is being conducted.



What happens during the trial?

If you consent to your child taking part in the trial, your child's medical notes, tests and scans will be reviewed by your child's medical team to make sure your child is eligible.

Once your child is registered on the trial and allocated a treatment plan, you will be asked to attend all of your child's scheduled clinic appointments, but you will also meet your child's research team who will need to know the following information:

- any medication your child is taking, including prescribed medication, over-the-counter medication and herbal medications/remedies
- any symptoms, side effects or complaints your child is currently experiencing
- any hospital admissions whilst your child is on trial treatment

Other things you or your child may be asked to do include:

- **Completing questionnaires**

These are important because they will tell the research team more about how the treatment is making your child feel and how it is effecting other parts of their life.

- **Providing extra samples of blood or urine**

Where possible, these will be taken at the same time that your child has routine procedures and blood tests. If additional procedures or visits to hospital are needed, this will be explained to you before you decide whether to take part in a trial.

It is important that this is not the main reason for deciding whether or not to take part. You can discuss with your child's research team a way in which treatments might be combined, or if there is any financial/transport assistance to help you attend these visits.

Taking part in a trial may last until the treatment ends, but often the trial will also include a follow-up period, so the research team will continue to record information relating to your child and their treatment. This will usually be at routine clinic appointments but sometimes you may be asked to visit the hospital specifically to provide this information. This will be explained to you before you agree to take part. Again, do not let this put you off taking part in the trial. Your child's research team will be able to talk through possible options to make sure this is not a burden to you.

You will meet your child's research team regularly during your child's time on trial. The results of your child's tests will be held in their medical notes as well as information about the treatment and any side effects. This information will be inputted into the trial database by the research team. Data about your child will always be anonymised when it is submitted to the trials database unless you are told otherwise.

How long do trials last?

This depends on the type of trial, and the number of patients needed to answer the trial question. Phase I and II trials usually last 1-2 years. Phase III trials may last a total of 5 years, or even longer. Often there is then a long period of follow-up. The length of time individual patients are on treatment within a trial will vary, but will be clearly spelt out in the trial protocol and information sheets.



What happens to my child's data?

All of the results from every patient who participated in the trial will be analysed. The information will show whether or not the new treatment is better. If it is, it will be approved for general use and will become the new standard treatment for future patients.

All patient information is kept secure and is covered by the Data Protection Act 1998. As it may be necessary to look back at information many years after a trial has ended, data are kept indefinitely either in paper form or digitally.

How can I find out about the results?

Some trials run for a considerable time. It is not possible to carry out the final analysis of the results until after the last patient has finished treatment and been followed up for a certain period. Trial results may not, therefore, be published until a few years after the last patient has finished treatment.

There is no standard way for patients to be kept informed about the research they have taken part in. Some clinical research studies have their own websites to keep patients and healthcare professionals updated about the study, and sometimes research teams have newsletters to keep participants updated about their study.

Most study results are published in scientific and medical journals. No individual patients are identified in these publications. These are written for doctors and often use quite complicated terminology. You can email the author (their details will be on the paper), who will be able to provide you with a simplified explanation.

Sometimes, a summary may be produced once the final publication is in print. Summaries of some previous trials are available on the Cancer Research UK Clinical Trials Database:

www.cancerresearchuk.org/about-cancer/find-a-clinical-trial

Additional sources of information

The parent/patient information sheets for a particular trial will give you more information about where and how the trial is run, and what it will mean if you decide to take part.

Reliable information about clinical trials is also available from the following organisations:

Cancer Research UK

Has an online database of cancer clinical trials, and reliable, easy-to-understand patient information.

www.cancerresearchuk.org

National Cancer Research Institute (NCRI)

The NCRI is a partnership between the government, charities and industry. The aim of the NCRI is to oversee and coordinate cancer research in the UK. The main research organisations map out what they are all already doing and plan jointly for future cancer research. This means that NCRI can identify gaps in research and monitor the progress that is being made across cancer research initiatives. They encourage collaboration between different research organisations to improve the speed and quality of cancer research in the UK.

www.ncri.org.uk

NHS Choices

A range of online information about clinical trials, including a video and links to national and international clinical trial databases.

www.nhs.uk

Nuffield Council on Bioethics

Nuffield Council on Bioethics provides an informative animation about taking part in research.

www.youtube.com/watch?v=6yaKwLG_vIE

Glossary

Data

This is individual patient information collected throughout the course of the trial. Analysis of the data allows conclusions to be formed about the results.

Chief Investigator

The lead clinician (doctor) for a clinical trial, across a number of sites or countries.

Ethics committee

The committee that reviews the protocol to ensure what is being proposed is ethical, safe and in the best interests of the patient. This committee will also review the information to be provided to the patient or parent to make sure they fully understand what they are being asked to consent to.

Inclusion and exclusion criteria

These are requirements that must be met by patients before being entered into a trial, for example, age, stage of cancer etc.

Principal Investigator

The lead clinician (doctor) at a particular site or treatment centre.

Protocol

The treatment plan that contains details of the purpose, design and conduct of the trial, and all the information on how doctors diagnose and treat the patient.

Randomisation

The random assignment of patients to different treatments within a clinical trial.

Regulatory body

The body that will approve the science of the trial and use of the drugs within the trial, and monitor the safety of the medicines being given.

Standard treatment

The best known current treatment.



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Children's Cancer and Leukaemia Group is a leading children's cancer charity and the UK and Ireland's professional association for those involved in the treatment and care of children with cancer. Each week in the UK and Ireland, more than 30 children are diagnosed with cancer.

We bring together childhood cancer professionals to ensure all children receive the best possible treatment and care. We fund and support research into childhood cancers, and we help young patients and their families with our expert, high quality and award-winning information resources.

If you have any comments on this booklet, please contact us. CCLG publications on a variety of topics related to children's cancer are available to order or download free of charge from our website.

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