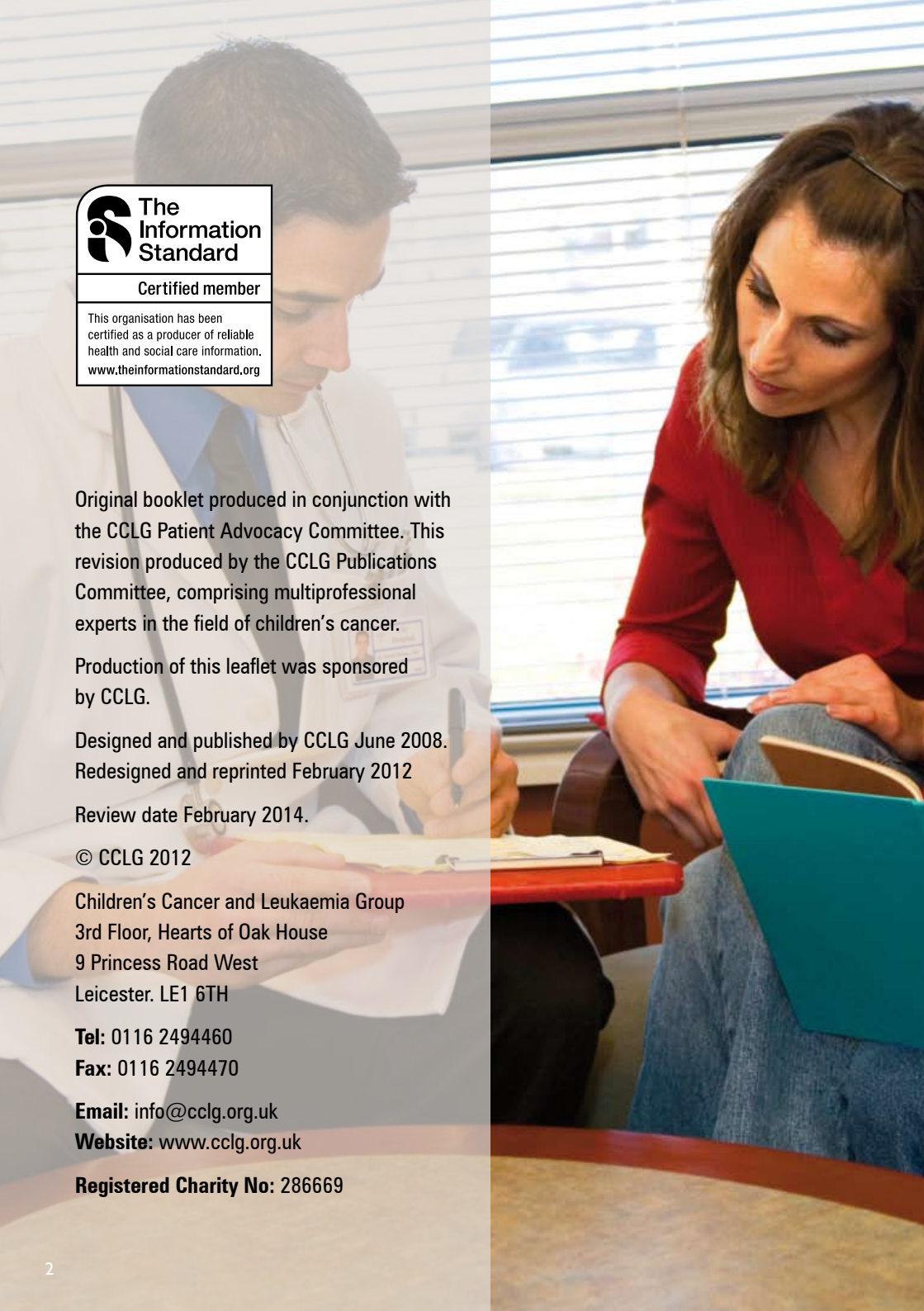
A Guide to Clinical Trials

For young people with cancer and their parents

Children's Cancer and Leukaemia Group – www.cclg.org.uk



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Many children and young people with cancer are treated on clinical trials. We hope this leaflet, designed for young people with cancer and their parents, will help you understand more about clinical trials, and answer some of the many questions you may have. Always discuss any questions or specific queries relating to treatment or participation in a trial with the doctor or other members of the team.

What is a clinical trial?

A clinical trial is a medical research study involving people. There are different types of trial. For example, a trial may compare one treatment against another, or be based on questionnaires to answer questions about quality of life. Clinical trials usually involve patients, but sometimes involve healthy volunteers.

Why are clinical trials important?

Because we don't yet know the best way to treat every type of cancer, clinical trials help us 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 possible 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 very small number of patients, usually with advanced cancer and who have had all available standard treatment.

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

Phase III trials aim to confirm the benefit of a defined treatment, often compared with the current or 'standard' treatment. These trials involve larger numbers of patients in real clinical care settings and usually run for much longer than phase I and II trials. They 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 trial. The inclusion and exclusion criteria (rules for being included) are clearly set out in the trial protocol (treatment plan). It is important that the patient is an exact match to these criteria, and that the patient or parents agree to take part. For example, the inclusion criteria may not allow all patients with a particular type of cancer to enter the trial, but just a specific sub-set of these patients, perhaps based on age or stage of disease.

How much will we be told about the trial and what it means to take part?

Detailed information sheets are provided for parents and patients, 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.

Does everyone in a trial get the same treatment?

Not necessarily. Some trials are known as randomised trials. In a randomised trial patients are randomly assigned to different treatments (known as treatment arms). The treatment assignment is usually done by computer, and each arm is a different treatment. This method means that neither the patient/parents, nor the doctor will be able to influence which treatment arm is allocated, and helps to ensure that the results are not biased in any way. Equal numbers of patients are treated in each arm and at the end of the trial the results are compared. Sometimes a trial may contain more than one randomisation. Your doctor will explain in more detail about how randomisation works, and precisely what it means in a particular trial.

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 that you agree to take part. This will be signed by either the parents or the patient themselves, depending on age.

How can we be sure the treatment is safe?

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. 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 efficacy of the treatment, the trial may be stopped.

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 that recruitment is completed as quickly as possible.

What if we say yes, and 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 doctor will respect your decision and you (or your child) will then receive the best known and proven treatment.

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

- You may receive a new treatment that is only available in a clinical trial
- Your treatment will be the same, wherever in the country you happen to 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 you will be monitored more closely than usual
- Sometimes there may be no benefit for you or your child but the results of the trial may help doctors improve cancer treatments for future patients.

Are there any disadvantages from taking part?

Your doctor will discuss with you any possible disadvantages. According to the design of the particular trial protocol, taking part may mean:

- You may have to make more hospital visits
- You may have more tests carried out
- The new treatment, although expected to be better, may not actually be better
- You 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.

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

The doctor treating you or your child will respect your choice and you/your child will receive the best known and proven treatment. Even if you agree to take part in a trial you can withdraw at any stage and you/your child will continue to be treated with the best possible treatment.

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 that individual patients are on treatment within a trial will vary, but will be clearly spelt out in the trial protocol.

Who is responsible for running the clinical trials?

Clinical trials in childhood cancer and leukaemia for patients in the UK and Ireland are run from a number of recognised clinical trials units, though increasingly nowadays they also involve collaboration with other international groups. The information sheets for a particular trial will give more details about who is running that trial. Occasionally trials may be run by a pharmaceutical company.

Where does the trial treatment take place?

There is a national network of treatment centres. These are where the vast majority of children and young people with cancer and leukaemia are treated.

What sort of information is collected?

The information (or data) collected on patients taking part in a trial includes details of diagnosis, treatment received, and also issues relating to any possible side effects. It may also be information about long-term follow up and quality of life.

What happens to the information?

Information on patients entered into a clinical trial will be collected in the treatment centre and sent to the trials unit responsible for coordinating the trial. The information will either be recorded on paper or sent electronically by computer. It will be stored in a secure database and then analysed by statisticians to provide information about the results of the trial.

How is patient confidentiality maintained?

All information about patients on trials is covered by the Data Protection Act 1998. This means that all staff who have access to this information are legally required to keep the information secure. This is very strictly governed and there are clear guidelines about disclosure of this information. Where possible, only patient initials or a patient code number will be used.

How long are trial data kept?

Because it may be necessary to go back and look at the information many years after the end of the trial, trial data are kept indefinitely, either in paper form or electronically.

Who monitors the way clinical trials are run?

Clinical trials are very closely monitored by a number of different individuals and organisations. This will be the Chief Investigator, 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. At a national level, there will be an ethics committee and the national regulatory body. If there are any concerns about the conduct of the trial or the results, a trial may be stopped early.

When are trial results available?

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

How can I find out the results?

The trial results are published in medical and scientific journals. These are written for doctors and often use quite complicated terminology. No individual patients are identified in these publications. It is not usual for trial results to be fed back to individual patients, but a summary may be produced once the final publication is in print. A number of summaries are available on the CCLG website (www.cclg.org.uk) or see the CancerHelp website (http://cancerhelp.cancerresearchuk.org/trials) .

Glossary of terms:

Data – this is sensitive 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 for a clinical trial, across a number of sites or countries.

Ethics Committee – the Committee that reviews the protocol to ensure that 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 at a particular site or treatment centre.

Protocol – a document 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 profile of the investigational medicines being given.

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 Help UK
Reliable easy to understand patient information from Cancer Research UK
www.cancerhelp.cancerresearchuk.org

NHS Choices
*A range of online information about clinical trials including a vid*eo.
www.nhs.uk

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If you have any comments on this booklet, please contact us at the address above.

CCLG booklets are available to download from our website.