Bloodwise

Your guide to clinical trials

For anyone affected by blood cancer

bloodwise.org.uk

PATIENT INFORMATION

Our patient information is for you and those close to you to use whenever, wherever and however you need it. You'll probably have lots of questions; this booklet aims to help you answer as many of them as possible.

A team of people helped produce this booklet. We'd like to thank Susan Neeson and Michelle Harry for their help and support in developing the content and checking for clinical accuracy.

Bloodwise staff revised the text to make it easy to read and a non-medical panel including people with blood cancer checked it for understanding.

The quotes in this booklet were contributed by people with blood cancer who have taken part in clinical trials.

A list of references used in this booklet is available on request.
Please email us at **information@bloodwise.org.uk**

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Introduction

This booklet is for people affected by blood cancer, who want to know more about clinical trials.

You'll probably have lots of questions; this booklet aims to answer as many of them as possible.

Our information is developed for and with people affected by blood cancer. It's written in line with national guidelines and created with healthcare professionals and specialist researchers, so you know it's accurate and up-to-date.

This booklet is one of many we make – you can find a list of our other booklets on page 38. For the very latest information, visit our website **bloodwise.org.uk**

Our booklets contain general information. Always listen to the advice of your specialist about your individual treatment – because every person is different.

When you see the symbols below in the booklet, it's a sign that we think the websites and other organisations mentioned will also give you good information and support.


Our website


Another of our booklets

Another page of the booklet


Another website


Another organisation

Contents

What are clinical trials?

All new drugs and treatments are thoroughly tested before they're made available to patients. Following tests in a laboratory, they're tested on people. Research studies involving testing new drugs and treatments on people are called clinical trials.

Clinical trials may test:

- new drugs that have passed safety studies,
- medical equipment,
- new combinations of current treatments, and
- different ways of giving treatments.

The goal of clinical trials is to improve treatments, quality of life and to find cures.

The clinical trial I was involved with gave me access to the best available treatment.

Why are clinical trials important to me?

Although the outcome for people with blood cancer continues to get better, there's still a lot more to be done to improve treatments and quality of life.

Clinical trials are really important, because they're the only way to develop new treatments – and improve existing ones – for you and other people with blood cancer. Researchers are able to compare the effects of new drugs and treatments to find out whether they work better than the current treatment used.

Clinical trials are also important to find out whether new treatments:

- are safe, and
- have side effects (and how to manage them).

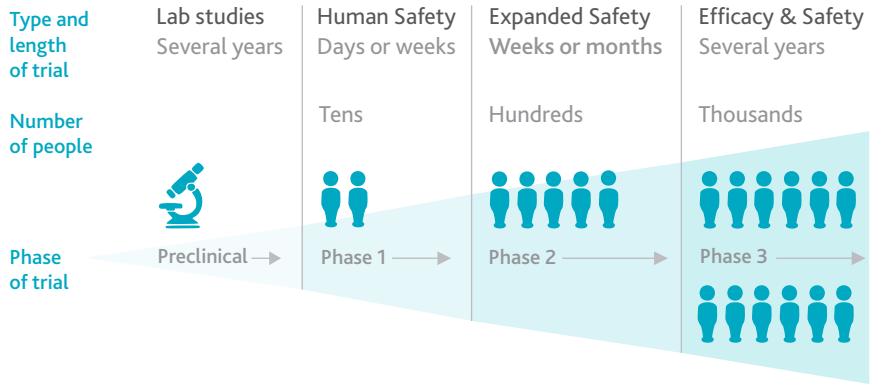
If clinical trials aren't carried out there's a risk that people could be given treatments that don't work and may even be harmful.

Clinical trials are divided into phases. The early phases look at the safety and the side effects of a new drug. Later phases test whether a new treatment is better than existing treatments.

An overview of clinical trials

What are the different types of clinical trials?

There are a number of phases that all new drugs or treatments must go through before they are made widely available.



Adapted with permission from the International Partnership for Microbicides

Phases of clinical trials

Phase 1

Phase 1 trials test the safety and side effects of a new treatment. People who take part in phase 1 trials (up to 30 people) often have advanced cancers, and have usually tried all the treatment options available to them. The first few patients to take part in the trial are given a very small dose of the drug. If they respond well, the next group have a slightly higher dose. The dose is gradually increased with each group. The researchers monitor the effect of the drug until they find the best dose to give. This is called a dose escalation study.

Phase 2

Phase 2 trials test the new drugs on a larger group of people (more than 100) to find out more about the side effects, safety of the new treatment, and the effects on the cancer. These trials can last a number of weeks or months, and sometimes they can continue for years if the person is responding to treatment.

Phase 3

Phase 3 trials involve more people (hundreds or thousands) and compare new treatments with the current treatment available longer-term.

Phase 4

Phase 4 trials are carried out after a new drug has been shown to work and has been licensed to be used (a licence means the medicine can be made available on prescription). These trials aim to find out how well the drug works when it's used on more people, the long-term benefits and risks, and more about the possible rare side effects.

Types of clinical trials

What are randomised trials?

If you take part in a clinical trial, you'll usually be put randomly into either:

- the treatment group – where you'll be given the treatment being trialled, or
- the control group – where you'll be given the existing standard treatment, or a placebo (a treatment that has no effect) if no proven standard treatment exists.

If you take part in a randomised trial (most phase 3 and some phase 2 trials are randomised) a computer programme is usually used to randomly select which group you will be in. Your personal details, such as age, sex and state of health, are taken into account so that the groups in the trial are as similar as possible. This helps to make sure that the results of the trial are reliable and accurate.

What are blind trials?

A blind trial is a trial where the people taking part don't know which treatment they are getting. You might get the new treatment. Or you could receive the standard treatment or a placebo. So that you can't tell which treatment you are having, everyone taking part will receive identical injections, tablets, or other form of treatment.

What are double blind trials?

In a double blind trial, you won't know which group you're in, and neither will the researchers, until the end of the trial. This makes sure that the results are more reliable as they will not be influenced by your expectations or the researchers' expectations.

What are open label trials?

In open label trials, both you and the researchers know the treatment you're having. These trials usually compare two very similar treatments to test which treatment is the best, and usually happen in the early phases.

All trials are checked and monitored to help keep you safe.

Setting up a clinical trial

How are trials funded?

Clinical trials in the UK are mainly funded by the government, drug companies and charities such as Bloodwise.

All trials, no matter who funds them, are checked and monitored in similar ways to make sure that everyone involved is protected. Each trial also has a sponsor who is responsible for running the trial. The sponsor may be the organisation funding the trial or the institution hosting the research (for example, a university or a hospital).

How are trials planned?

Once a group of researchers have an idea, they need to develop a protocol. A protocol is a detailed plan for the trial, which includes:

- why the trial should be done,
- the number of people involved,
- who should be able to take part in the trial (eligibility),
- details of the treatment given in the trial, and
- what tests people will have and when.

Everyone involved in the trial has to use the protocol. This keeps the people taking part safe and makes sure that the results of the trial are reliable.

If you see a trial that you are interested in, you need to discuss it with your doctor or cancer specialist.

Joining a clinical trial

Where can I find a clinical trial?

Talking to your specialist

If you want to join a clinical trial, you usually have to be referred by your doctor. Your doctor will have all your test results, records and reports so they'll know whether a trial is right for you.

There may be a trial locally that would suit you. Your cancer specialist will also know about any large national or international trials for your type of cancer and will be able to tell you if they're suitable for you.

Talk to us

We invest a lot of our money in funding clinical trials. You can find out which trials we are funding here:

bloodwise.org.uk/page/our-clinical-trials

0808 2080 888

"Taking part in a clinical trial gave me a sense of doing something positive to fight against my cancer."

Databases and organisations

You can also search several online databases and organisations to find a clinical trial and more about:

- where it's carried out,
- who to contact,
- if it's still recruiting people, and
- who is eligible to take part.

Here is a list of some online databases and organisations:

Information from the NHS about clinical trials
- nhs.uk/conditions/clinical-trials

The Medical Research Council (MRC) Clinical Trials Unit
- ctu.mrc.ac.uk

National Institute for Health Research UK Clinical Trials Gateway
- ukctg.nihr.ac.uk

The U.S. National Institutes of Health
- clinicaltrials.gov

Opportunities for public involvement in clinical research run by the National Institute for Health Research
- peopleinresearch.org

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

Current controlled trials on the ISRCTN registry
- controlled-trials.com

Can anyone join a trial?

All trials have guidelines about who can and can't take part.

These guidelines are called eligibility criteria. Eligibility criteria are used to make sure that the trial:

- includes people who may benefit from the treatment, and
- makes sure that people are not taking an avoidable health risk.

Some entry criteria will be communicated to you before you enter a clinical trial. Others will not, and may include pre-trial tests, which can only be carried out once you've agreed to enter the trial.

What if I don't meet the trial criteria?

It's important for your own safety and the success of the trial that you meet all of the trial criteria. Some trials may only want people who are in a particular age group, a certain sex, or have a particular stage of cancer.

You may be very upset and disappointed if you don't meet the entry criteria of a trial, especially if you do go through the pre-trial tests. If you can't enter the trial, your doctor or research team will talk through any alternative treatments available to you or other potentially suitable clinical trials.

If you see a trial on a database that you're interested in, you need to discuss it with your own doctor or cancer specialist. To help you, you could print off a copy of the information and take it to your appointment.

Generally, having a medical referral is the only way you can join a trial. You should talk to your own doctor first, rather than trying to contact the people running the trial. They won't be able to sign you up to the trial without your doctor's input.

You could also make a note at the back of this booklet, on **page 32**. If the trial looks suitable for you, your doctor will contact the trials team to ask if you can take part. This is called a medical referral.

Giving your consent

Consent is when you give your permission to have any medical treatment, test or examination.

Before giving your consent, you'll be given a patient information sheet, which includes detailed information about the trial to help you make a decision and, later, to refer back to.

Once you've decided to take part in a clinical trial, you'll have to give your informed consent and sign some forms.

These forms are important legal documents that protect both yourself and the organisation organising the trial.

Before you give your consent, your trials team will discuss the trial in detail with you, the tests you may need, the frequency of your hospital visits and any known benefits, risks and side effects of the treatment. They will also discuss alternative treatments and options with you so you know all of the options before making a decision. It's important that you ask any questions that you might have.

"I was really honest and said, 'I have no idea what this means,' so my consultant discussed the trial with me, which was great because he encouraged me to ask the questions that I probably thought were stupid."

How do I decide if I would like to take part and how long do I have to decide?

There's no pressure for you to make a decision right away. It's important to take as long as you need. You should always discuss your options with your family, friends and doctor. This is to make sure that all of your questions have been answered and you understand the information that's been given to you.

What if I change my mind?

You can leave a trial at any point without giving a reason. But if you're happy to give a reason, it could help the research team design better trials in the future. If you do change your mind, your decision will be respected. You'll still be offered the standard treatments for your stage of cancer, however, your treatment options may be affected if you've been having a certain type of treatment during the trial which could affect the standard types of treatments.

If you want to find out more about which groups check the safety and standards of a clinical trial visit:

The Medicines and Healthcare products Regulatory Agency
> **gov.uk/government/organisations/medicines-and-healthcare-products-regulatory-agency**

Research Ethics Committees
> **hra.nhs.uk/about-us/committees-and-services/res-and-recs/**

Will I be safe?

Every new treatment carries some risks, but the overall aim of a trial and the researchers is to reduce these risks for you. If at any stage the risks of the trial are greater than the benefits for you, the trial will be stopped.

To keep you safe, the trial is monitored by the following groups.

Medicines and Healthcare products Regulatory Agency (MHRA)

The MHRA is a group of the Department of Health that makes sure the safety and quality of the treatment tested in a trial meets good practice standards.

Research Ethics Committees

Research Ethics Committees are groups run by the NHS Health Research Authority. They are independent groups of people including doctors, nurses, other medical staff, members of the public and sometimes lawyers. The group makes sure that your wellbeing and rights are upheld. They also make sure that the information you are given tells you everything you need to know and is easy to understand.

Clinical trial/research committee

These are national and local groups in your hospital or research centre that make sure that the trial is planned well and carried out safely. They also check that the trial is based on scientific knowledge and follows national guidelines and safe practices.

Who will have access to my medical records?

The NHS and the sponsor (the organisation running the study) have strict confidentiality rules about who can and can't view your information. Only the clinical and research team who need to access your records will do so. Once you've agreed to take part in a trial you'll be assigned a trial number. If your information needs to be sent out of the hospital the trial team will use this number only and all of your personal details will stay in your notes.

An expert team of researchers will be involved in your trial and will be there to answer any of your questions.

Being on a clinical trial

There are always risks involved in a clinical trial. But the trials are planned to be as low-risk as possible while making the benefits as great as possible for anyone who takes part.

What are the possible benefits of being on a trial?

The main advantage of being on a clinical trial is access to promising new treatments which aren't currently available on the National Health Service (NHS). However, researchers can't guarantee that all people taking part in trials will get a treatment that works. And it's important to know that new treatments may not always be better than the current treatment.

> *The close monitoring was very reassuring.*

Other benefits can include:

Close monitoring
You may have more tests and appointments on a clinical trial than you normally would in standard care. Some people find this reassuring.

Specialist staff
An expert team of researchers and clinicians will be involved in a clinical trial and they will be happy to discuss any worries and fears that you may have. During a trial, you're also likely to have contact with a clinical nurse specialist (CNS). The CNS will provide you with information about the trial and answer any questions you may have. They're there to support both you and your family.

Helping others
Sometimes clinical trials won't benefit you directly but the information collected can play a very important role in finding future treatments and helping others. People who take part in trials often feel that they're taking an active part in their healthcare.

What are the possible risks of a trial?
You should be aware of the possible risks of being on a clinical trial. These can include:

Time and money
You will have to attend hospital more often, which could cost time and money. Some clinical trials will offer to pay for your travel expenses – this should be outlined in your patient information sheet.

Worry and concern
As the treatment is part of a clinical trial, there are never any guarantees that the treatment is better than the current standard treatment.

Lots of information to process
In order to make sure that you fully understand what you're agreeing to, the trials team have to provide lots of information about the trial, side effects and other treatment options. Some people may find taking in lots of information difficult and overwhelming. You may find it helpful to take someone to your appointments for support or to make notes to read later.

Extra hospital visits and tests
If you take part in a trial you'll be monitored regularly during and after the study. You'll have regular tests and you may be asked some extra questions about how you're feeling. Sometimes this means going to your hospital or GP more often than you would normally, so keep this in mind before you agree to take part. Ask your trials team how many extra visits will be needed and consider how convenient this will be for you.

Unknown side effects
As the drug or treatment is still in the testing phase, the trial team may not fully know what the potential side effects may be. You and your healthcare team will need to balance the possible benefits of being on a trial against the risks of short and longer-term side effects.

You'll have to attend important follow-up appointments after a trial to see how you're feeling.

After a trial

What follow-ups will I have?

Researchers will follow your progress for some time after treatment. They do this to see if the treatment works over a longer period of time and to find out more about the long-term side effects.

Follow-up periods can range from a few months to more than 10 years, depending on the type of treatment and people involved.

Trials that test if a new treatment is better at stopping cancer coming back or trials looking at preventing cancer will need to be followed up for several years to get accurate results.

When will the trial end?

You will stay in the trial until one of the following happens:

- the trial comes to an end – as defined in its protocol,
- if the treatment is failing or there are safety concerns,
- if your doctor believes it's in your best interests to take you off the trial, or
- you decide to withdraw.

What happens at the end of a trial?

Some trials can run for many years so it may be some time before the results of a trial are known. At the end of a trial the results will be available to everyone who took part.

You should keep in mind that the results of the whole trial will not affect your personal reaction to the drug or treatment.

They will also be published so that others can use the information to help them make decisions about their treatment and healthcare.

The researchers must publish the results, regardless of what they show, and state how the results add to medical knowledge.

If you're having a new treatment as part of a trial, you may not always be able to continue on this treatment when the trial ends. It may be some time before a new treatment is provided by the NHS. In this case you will be given the standard treatment.

Many of the organisations that fund clinical trials involve people to help them decide what will be researched in the future. It's essential that researchers listen to the needs and interests of the people they're trying to help.

After you've finished taking part in a clinical trial, you may be able to take part in another one. However, you should always check with your doctor first.

Bloodwise is committed to listening to people with blood cancer and their families. You can read about our current research here > **bloodwise.org.uk/our-achievements**

Through trials, we can develop new treatments and improve existing ones. A large part of our research investment each year is devoted to funding clinical trials.

Bloodwise's involvement in clinical trials

Our research impact: past and present

Bloodwise is committed to improving the survival rate and quality of life for people with blood cancer. Our work started in 1960, when the Eastwood family founded our charity following the death of their daughter Susan, who died from childhood leukaemia. At that time, the survival rate for children with ALL was less than 1 in 10. Our research over the years has helped to increase that to 9 in 10.

Since then, a large part of our research each year is devoted to funding clinical trials, with £7.5 million currently committed to helping fund 35 trials.

Our researchers have made – and continue to make – crucial discoveries to improve treatments, survival rates and prevent cancer from happening in the first place.

For more information about Bloodwise's involvement in clinical trials go to > **bloodwise.org.uk/our-clinical-trials**

What is the Trials Acceleration Programme (TAP)?

The Trials Acceleration Programme (TAP) is Bloodwise's investment in the development of clinical trials in blood cancers for the UK. The programme supports quick access to world-class clinical trials in blood cancer.

A central hub at the University of Birmingham coordinates 13 trial centres at hospitals around the UK. This means **22 million people** in the UK have access to the very latest promising blood cancer treatments.

So far, 18 new clinical trials have been approved thanks to TAP - and that figure's growing all the time.

TAP is an example of how we work collaboratively with other organisations to achieve the best outcomes for those with blood cancer. We have just renewed funding for our TAP centres and have committed funding of nearly £2 million to the centres for the next three years.

For more information about TAP please go to >
bloodwise.org.uk/page/our-trials-acceleration-programme

Notes

Notes

About us

We're Bloodwise, the UK's specialist blood cancer charity.

We're here to make things clear

We send our patient information for free to anyone who needs it. Whether you have blood cancer yourself or care for someone with blood cancer, we have a range of booklets, fact sheets and online information to support you and help you make sense of it all.

We're here to listen, support and connect

Our Support Line team are just a call or email away. Call us on **0808 2080 888** Mon–Fri 10am–4pm, email **support@bloodwise.org.uk** or visit us at **bloodwise.org.uk** to join our online community.

We're here to beat blood cancer

We fund the research that gets results: research that tells us more about blood cancer and improves the lives of those with blood cancer. We've invested over £500 million in world-class research since 1960 – but we won't stop until every single person with blood cancer can live their life to the full.

Go to > **bloodwise.org.uk** for more information

Getting involved

Help us beat blood cancer.

We have lots of exciting opportunities for you to get involved and help us to beat blood cancer.

Give a gift

Whether it's a regular or one off donation, every gift – big or small – will make a difference.

Take on a challenge

Every stride, stroke and pedal gets us closer to beating blood cancer. Whatever the event, make every mile matter.

Beat blood cancer locally

Join one of our regional branches and fundraising groups to discover how you can make a difference.

Jump online

Every like, share, tweet and mention could mean someone finds out about our services and raises awareness of blood cancer.

facebook.com/bloodwise.uk
twitter.com/bloodwise_uk

Partner with us

We're always looking for companies who share our vision and energy.

Get in touch

Visit **bloodwise.org.uk/get-involved**, call **0207 504 2200** or email **hello@bloodwise.org.uk**

Your feedback

We're always looking for ways to improve the information we provide for people with blood cancer.

We welcome your feedback on this booklet and our other patient information. Any improvements you suggest mean we can make better information for people with blood cancer and those close to them.

Email us at **information@bloodwise.org.uk** with your feedback.

More information

We offer patient information on many blood cancer types and topics, online and in free printed booklets.

They cover everything from symptoms and diagnosis through to treatment and living with your condition.

For our patient information, go to >
bloodwise.org.uk/information-and-support

Information booklets

Booklets which are available free of charge:

Reference	Description
	Leukaemia
BWALL	Acute lymphoblastic leukaemia (ALL)
BWAML	Acute myeloid leukaemia (AML)
BWAPL	Acute promyelocytic leukaemia (APL)
BWCLL	Chronic lymphocytic leukaemia (CLL)
BWCML	Chronic myeloid leukaemia (CML)
	Childhood leukaemia
BWCHALL	Acute lymphoblastic leukaemia (ALL) in children and young adults up to 16 years
BWCHAML	Acute myeloid leukaemia (AML) in children and young adult up to 16 years
	Lymphoma
BWHL	Hodgkin lymphoma (HL)
BWNHLHIGHGRADE	High-grade non-Hodgkin lymphoma (NHL)
BWNHLLOWGRADE	Low-grade non-Hodgkin lymphoma (NHL)
	Other
BWMM	Myeloma
BWMDS	Myelodysplastic syndromes (MDS)
BWMPN	Myeloproliferative neoplasms (MPN)
	Treatment and beyond
BWCT	Your guide to clinical trials
BWSEVEN	The seven steps: blood stem cell and bone marrow transplants
BWDAPN	Eating well with neutropenia
BWMYDIARY	Diary for anyone affected by blood cancer

Fact sheets

We have the following fact sheets available online at **bloodwise.org.uk/information**

- Blood transfusions
- Burkitt lymphoma
- Chronic myelomonocytic leukaemia (CMML)
- Hairy cell leukaemia (HCL)
- Large granular lymphocytic leukaemia (LGLL)
- Monoclonal gammopathy of undetermined significance (MGUS)
- Mucositis
- Plasma cell leukaemia
- Solitary plasmacytoma
- T-cell acute lymphoblastic leukaemia (T-ALL)
- Transformation of chronic lymphocytic leukaemia (CLL)
- Treatment decisions
- Understanding infection
- Waldenström macroglobulinaemia
- Watch and wait
- What to expect from your appointments

More information from Bloodwise

You can order more information by:

- visiting **bloodwise.org.uk/information**
- emailing **information@bloodwise.org.uk**
- calling **020 7504 2200**
- or completing and sending this form to us freepost using the address:
  FREEPOST PLUS RTSU-XAYE-X2YK, Bloodwise, 111 George St, Edinburgh, EH2 4JN

All of our information is free to people affected by blood cancer, but if you would like to include a donation with your order, please fill in the donation form over the page.

Please send me some information

Title ..... First name ..... Surname .....

Address .....

Postcode ..... Email ..... Phone .....

Please write the reference codes of the booklets that you would like to be sent to you (free of charge) in the spaces provided below:

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Keep in touch

We'd love to keep you updated about our exciting work and the ways you can help, including campaigns and events that you might be interested in. We promise to respect your privacy and we will never sell or swap your details.

I am happy for Bloodwise to contact me by: ☐ Email ☐ Phone ☐ SMS

☐ Please don't contact me by post

You can change how we communicate with you at any time.
Contact us on **0808 169 5155** or email **hello@bloodwise.org.uk**

Bloodwise. Registered charity 216032 (England & Wales) SC037529 (Scotland) Ref:PINFO

My details

This is a place to put important information about you, your condition and key contacts.

My name and hospital number

My NHS number

My condition

My contacts

My consultant

My key worker (usually CNS)

Haematology ward

Haematology clinic

Other contacts

Bloodwise

The **blood cancer research** charity

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bloodwise.org.uk
020 7504 2200 (Reception); 0808 2080 888 (Support Line)

Registered charity 216032 (England & Wales) SC037529 (Scotland)
CT/0317.1