

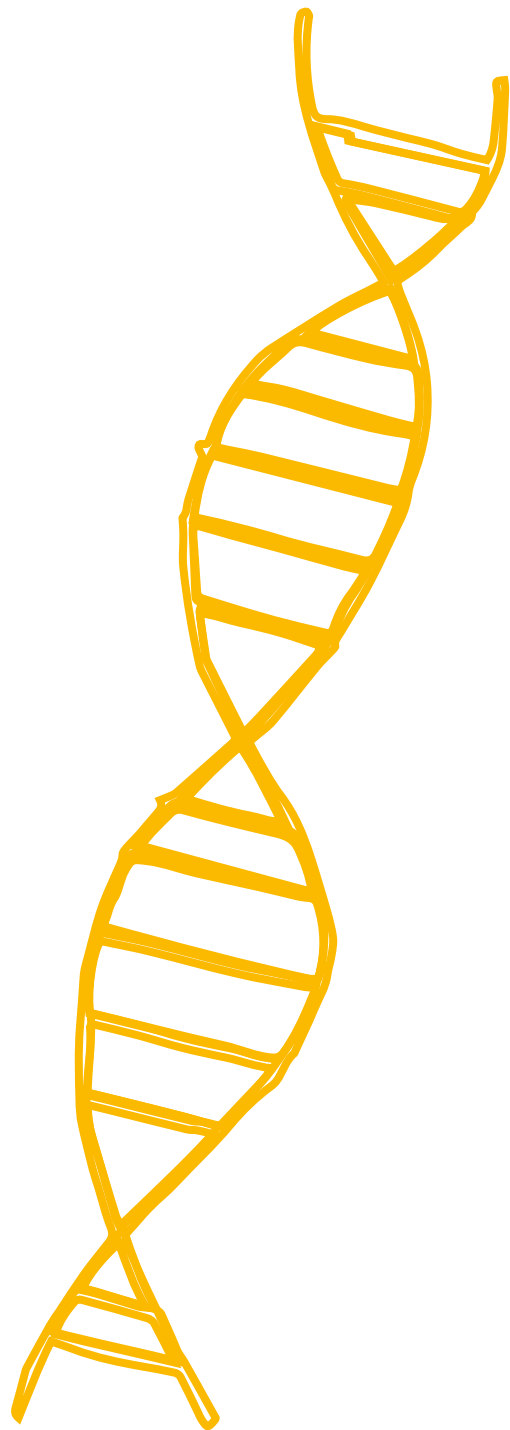
Cystic

**Fibrosis advancing
care with clinical trials**



Taking part in Clinical Trials:
A guide for people with cystic fibrosis,
parents and family members

Fighting for a Life Unlimited



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Letter from Oli

To everyone living with cystic fibrosis,

In recent years, significant advances have been made in the understanding of cystic fibrosis (CF) and the development of new treatments addressing the root cause of the condition. In the 1960s, CF was a childhood condition; today, there are more adults than children living with CF, and the outlook continues to improve. Much of this has been made possible by clinical trials.

New therapies that target individual mutations in CF, known as ‘precision medicines’, are showing real promise in halting the progression of lung damage. A number are in the pipeline, including some which could treat the most common mutations found in CF, but also some targeting rarer mutations. In addition, research in the UK and internationally is seeking to address some of the key medical challenges in CF, such as lung infections, CF-related diabetes, CF-related liver disease and management of digestive issues.

Without clinical trials, the CF treatments we use would not exist. Enzyme supplements such as Creon, antibiotics including Ciprofloxacin and Tobramycin, treatments like DNase and hypertonic saline that make it easier to clear mucus from the airways and, more recently, precision medicines such as Kalydeco – each of these has had a huge impact on care and treatment for people with CF and led to improvements in quality and length of life. Even equipment, nebulisers and PEP masks for example, have to be tested to make sure they are safe and effective. We benefit from these treatments because people with CF, young and old, and their families, clinical teams and researchers have been involved at some level in the clinical trials process. It’s important that we build on this momentum.

Clinical trial participation can seem like a big unknown. This booklet aims to provide clear information about clinical trials in cystic fibrosis. As well as information about how trials work, and practical considerations, I encourage you to read the case studies of people with CF who have participated in a variety of trials, speaking openly and honestly about their experience and what it meant for them.

Clinical trials cannot happen without us - people living with CF - choosing to take part.

We hold the key to better treatments and we have the power to accelerate their development. Working in partnership with CF centres and people living with CF, the Cystic Fibrosis Trust has developed the Clinical Trials Accelerator Platform (Trials Accelerator) to attract more trials to the UK, share information about them and give people with CF across the UK a fairer opportunity to access them.

I am excited about this community-wide effort to accelerate clinical trials, with the potential to improve the quality and quantity of life for people living with this condition. As someone who has lived with CF for over 40 years, I find it heartening to know that the Trials Accelerator is part of a coordinated global effort to develop better treatments for people with CF as quickly and efficiently as possible.

I hope this booklet will help answer some questions you may have about clinical trials, encourage you to talk to your CF team about opportunities to participate and help you make decisions that are right for you.



Oli Rayner, (age 42)



What are clinical trials?





"I felt good being part of something so important."

Martin, (age 43, adult with cystic fibrosis)

What are clinical trials?

A clinical trial is a type of project designed to investigate the effect of a treatment (or intervention) on human health. Interventions may be new medicines or medical devices, but may also be behavioural or lifestyle changes, such as modifying diet or physical activity. Clinical trials are designed to investigate whether the intervention is both safe and effective.

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The National Institute for Health Research says...

Clinical trials are the best way to compare different approaches to preventing and treating illness and health problems. Health professionals and people with CF need the evidence from trials to know which treatments work best. Without trials, there is a risk that people could be given treatments which could be harmful or have no benefit.

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Without clinical trials, treatments for conditions, from CF to cancer, to diabetes, would never have been developed. Clinical trials are the only way to ensure that new treatments are safe and effective.

All your prescribed treatments will, at some stage, have been through a clinical trial – so they really matter to everyone, including people with cystic fibrosis.

Participation in clinical trials can be very rewarding and people with CF have told us some of their reasons for taking part:

- Understanding your condition better
- Taking a more active role in your healthcare and decisions that affect your treatments and quality of life
- Helping researchers to develop new treatments to benefit future generations
- Benefiting from regular follow up meetings when involved in a clinical trial. This can mean that health problems may be found earlier
- Receiving treatments that are not yet widely available
- Helping others with cystic fibrosis

However, it's a personal decision and you need to be sure you have all the information about the trial you're interested in so that you can make the right decision for you.

How do clinical trials work?

Clinical trials in the UK are tightly regulated, and there are a number of requirements that must be met before a trial is allowed to go ahead. Trial organisers must obtain authorisation from independent bodies such as the Medicines and Healthcare Products Regulatory Agency (MHRA) and Health Research Authority (HRA) before trials of new medicines can take place. These bodies examine every aspect of the trial documentation to make sure it's ethical, well-organised and safe. They also ensure any potential risks to participants are minimised.

Trials may be instigated by pharmaceutical companies who want to investigate the safety and effectiveness of a new treatment they have developed, or by healthcare professionals or researchers who want to answer specific questions about a treatment.

Clinical trials can be designed in different ways; controlled trials, randomised controlled trials and observational studies are all ways of investigating the impact of a treatment. Please visit cysticfibrosis.org.uk/Get-involved/Clinical-trials/Taking-part-in-clinical-trials for more information on clinical trial designs.

Most clinical trials looking at new treatments are controlled (comparative) – essentially they compare standard treatment or no treatment with a new intervention.

The earliest example of a controlled trial is thought to be from the 1800s when ship's doctor James Lind separated his scurvy-suffering crew into groups; some receiving the 'treatment' of lemons while others received various additions to their diet including vinegar and salt water. Very quickly, James Lind noted his treatment group fared significantly better!



A randomised controlled trial (or RCT) is a controlled clinical trial as described above, but in this case participants are randomly assigned to either the treatment or control group. In some RCTs, the participants won't know if they are receiving the new treatment or if they've been allocated to the control group where they will receive existing treatment or a placebo.

Some research studies may simply be observational. Observational studies aren't designed to investigate a new intervention or treatment but instead are looking at different variables (such as mood or FEV1) over a period of time.

"Over the years I have been involved with many trials as I feel it is the least I can do - it's my opportunity to pay back for future generations"

Jane, (age 53, adult with cystic fibrosis)

Did you know?

A placebo is an inactive substance, usually a capsule, pill or liquid, used in clinical trials to compare against a new medication drug. It is identical in appearance to the medication being studied but it has no medicinal action and is usually made of starch, sugar or salt.





I have always been very interested in clinical trials, and have had the opportunity to participate in several clinical trials. The first was trialling the hormone Grehlin. If I'm honest, this trial was pretty hard. I didn't feel I had all the facts to hand before I took part.

My second trial was much easier and I felt much more informed. I took part in a home monitoring study; I monitored my lung function, weight, sputum and oxygen levels and uploaded these by phone. I wore a fitness tracker to measure daily movement and I also recorded how I was feeling.

This helped me become much more aware of my own health, which I found very empowering. It didn't impact on my day and I found myself trying to beat my own results!


























I'd encourage people to be proactive and speak to their CF centres if they want to find out what trials are available to them. I think clinical trials give people the power to be in charge of their own health – a chance to help themselves and others, especially those who cannot participate themselves.

My experience of clinical trials has made me more in tune with my own health, and overall that is something I have found very engaging.

Cleo, (age 32 adult with cystic fibrosis)



Phases of clinical trials

Phase	Length*	No. of Participants		Purpose
 <p>Phase 1</p> <p>Small study, less than 30 people. Looks at whether a trial treatment is safe or has side effects.</p>	 <p>1 month</p>	 <p>10 - 20</p>	 <p>Is it safe?</p>	 <p>How does the body process it?</p>  <p>What are the side effects?</p>
 <p>Phase 2</p> <p>Larger study. Looks at how well a treatment works.</p>	 <p>3 - 12 months</p>	 <p>up to 100</p>	 <p>Is it safe?</p>	 <p>How well is it working?</p>  <p>How much should be taken?</p>
 <p>Phase 3</p> <p>Much bigger, possibly hundreds. Tests new treatment against standard existing treatment.</p>	 <p>6 - 24 months</p>	 <p>100 - 300</p>	 <p>Is it safe?</p>	 <p>How well is it working?</p>  <p>Does the benefit outweigh the risk?</p>
<p>If successful Application submitted to NICE</p> 				
 <p>Phase 4</p> <p>Further assessment and monitoring of treatment after it has been licensed.</p>	 <p>6 - 24 months</p>	 <p>100 - 300</p>	 <p>Does it still appear to be safe?</p>	 <p>Are there rare side effects?</p>  <p>Cost effectiveness & comparison to other similar medicine</p>

 = 15 participants with CF  = Number of months of participant involvement

*number of participants and length of study varies based on study characteristics



How do I take part?





How to take part in clinical trials?

Thinking about taking part in a clinical trial is a big decision, and it's important that you have all the information you need to make an informed decision.

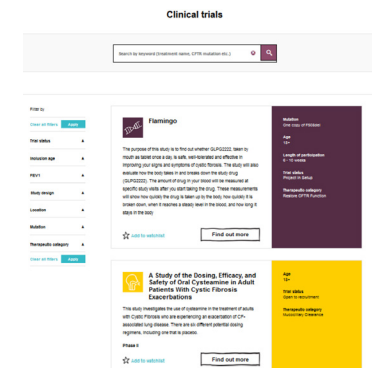
It might be helpful to start by finding out what trials are taking place, and there are a number of ways you can do this.

Clinical Trials Digital Hub

The Cystic Fibrosis Trust has developed a Clinical Trials Digital Hub as an interactive way to inform and educate people about cystic fibrosis clinical trials, using real life examples of clinical trial participation and answering frequently asked questions about participation.

As part of the Clinical Trials Digital Hub, the Cystic Fibrosis Trust has created the UK's only CF-specific clinical trials database, the CF Trials Tracker. This database lists all CF clinical trials open for recruitment with details about each trial and who to contact for more information.

Please visit cysticfibrosis.org.uk/trialtracker to find out more or head over to cysticfibrosis.org.uk/takingpart to learn more about taking part in clinical trials.



Your CF team

Your CF team may already be proactive in discussing clinical trials with you. If not, raise it and let them know that you would like to be kept up to date with what trials are taking place, and potential opportunities to participate, whether locally or at other CF centres you may be willing to travel to.

You may have found something of interest on the Trials Tracker to start this conversation.

"I've always been very interested in clinical trials and asked my CF team to let me know if there were any trials I could take part in."

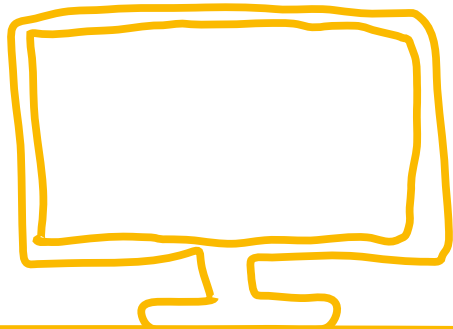
Cleo, (age 32)

Remember that just having a discussion about taking part in a trial doesn't mean you are obliged to take part, and you should never feel under any pressure to do so. Whether or not you decide to take part will have no impact on the care you receive from your CF centre or clinic.

You can also withdraw from a trial at any time, without giving any reason or it affecting the regular CF care you receive.

"I had been bugging my CF consultant for a while to see if there were any trials I could take part in."

Simon, (age 35, adult with cystic fibrosis)



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Over the years I have been involved with many studies, surveys and trials. I have undoubtedly benefited from advances in CF care brought about by clinical trials, and I feel it is the least I can do to give something back.

I have been lucky to have a CF consultant who was particularly focussed on CF research, and have always made it known to my CF team that I was keen to participate in clinical trials.

I was a case study for the TOBI podhaler in December 2011, a treatment which is in use today. In the mid-'90s I took part in the Ciprofloxacin trial – then a new antibiotic, it is also now in everyday use over 20 years later.

More recently, I was fortunate enough to meet the criteria for the Kalydeco trial, having one df508 mutation and S497R. I am now on the full dose of the drug and my lung function is over 90%.

I've been lucky to have benefited from my own participation in trials and from wider developments in CF medicine. My further participation in trials, as well as fundraising for research, feels like I can continue to help future generations. I was incredibly proud to be invited as a keynote speaker to talk about my experiences at the Cystic Fibrosis Trust's 50th Anniversary Gala, which poignantly coincided with my own 50th birthday.

Jane, (age 53)



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Can I take part?

Anyone can potentially take part in a clinical trial, but whether or not you are suitable will depend on the eligibility criteria for each specific trial.

A screening visit will help determine if the trial is suitable for you by checking your health and medical history against the inclusion and exclusion criteria. The screening process should be fully explained to you beforehand and will not take place until after you've given your consent to take part in the trial.

Eligibility criteria vary from trial to trial and are typically based on characteristics such as:

- Age
- CF genotype
- Severity or stage of illness
- Pregnancy
- Treatments currently in use
- What other medical conditions may be present

Being told you're not eligible to take part in a particular clinical trial can understandably be very disappointing, however you may find you are eligible to participate in a different trial. It's really important that clinical trials have strict eligibility criteria in place to

make sure the trial is conducted as safely and accurately as possible.

"I've been approached by my team about a few trials over the years. However, for various reasons, none have been suitable. I underwent pre-assessment for Orkambi, but my health wasn't stable enough to go on the trial for the drug. For other trials, there were certain criteria I didn't meet. It's disappointing, but understandable."

David, (age 33, adult with cystic fibrosis)

As trial participation is often staggered over a period of time, not meeting the inclusion criteria at the start of a trial might not necessarily exclude you from taking part later on, as health characteristics such as your lung function may change over time which could lead to you meeting the inclusion criteria in the future.

"I spoke to the trials coordinator at my hospital about taking part in the Orkambi combination therapy trial. I didn't get on the trial at the first screening due to my health being unstable and the treatments I was on, meaning I didn't meet the criteria. I was disappointed, but knew it was a long shot. I will retry for a second screening in future though."

Lee, (age 27, adult with cystic fibrosis)

Should I take part?

Deciding whether or not to take part in a clinical trial is a personal decision; you should never feel pressured to take part. People with CF who have participated told us that it's really useful to get as much information as possible about the trial, so you can make an informed decision.

You should take as much time as you need to decide whether or not to take part. You may also find it useful to talk about it with your family and friends, especially any possible impact on your personal life, work/school and family commitments.

It is important to be aware that you would not necessarily receive a new treatment by participating in a trial – you may receive standard treatment or a placebo, but this still often contributes to a positive experience by helping identify the best treatment for yourself and others.

"Being involved gave me a feeling of worth."

Sammie, (age 35, adult with cystic fibrosis)

You have to weigh it up for yourself – consider all the pros and cons. Never be afraid to ask questions of your team. But if you do decide to take part, do it for the right reasons. Respect it and give it what it deserves.

Simon, (age 35)



Informed Consent

Informed consent is a process to make sure that potential participants in clinical trials:

- Can ask questions and get answers before, during and after the trial
- Fully understand the nature and purpose of the trial
- Fully understand what the trial will involve
- Are informed about potential benefits to health, risks and inconveniences
- Only agree to take part willingly
- Remain informed throughout their period of participation

This process starts when your CF doctor or researcher introduces details of the clinical trial to you. Following initial discussions, you will be given information to take home to read and consider further with family or friends, who may also attend meetings with you. You should feel confident that you have enough information to help you decide whether you want to take part in the trial, and that you have been given enough time to consider all the information and what the trial will mean to you in practical terms, such as extra appointments and tests.

You can't be entered into a trial without giving your written consent. Where clinical trials involve children the consent process is also different and will be fully explained by the CF doctor or researcher you talk to about the trial.

Find out more about informed consent in both adults and children by visiting cysticfibrosis.org.uk/takingpart

If you decide to take part, you will be given a consent form to sign to say that you understand the information and give your consent willingly – you should retain a copy of this form for your own records.

Informed consent is an ongoing process throughout your period of participation in a trial. Researchers should continue to provide information, answer your questions and advise if any new relevant information comes to light during the trial. You can reconsider your participation at any point, or even withdraw without the need to give a reason. Your decision must be respected and will not affect your ongoing CF treatment plan.

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I have taken part in a number of clinical trials, and have always made sure my CF team were aware of my interest in taking part. I am quite regimented about sticking to my treatment so I think my CF team views me as a good candidate!

I took part in a trial for DNase when I was a teenager. The DNase trial showed the treatment was effective, and I'm still benefitting from it today. Now I want to do all I can to help the younger generations living with cystic fibrosis.

My experiences of taking part in clinical trials have been largely positive, and I think there are a lot of advantages in taking part. Often, you will be seen by the best expert clinicians and researchers, and much closer attention will be paid to your overall health. It's a chance to learn much more about your CF and become more expert in your condition. I also quite enjoyed the novelty of attending clinic out of choice rather than necessity!

Where I can't participate, I can still get involved in clinical trials. Feedback from the CF community will help to set achievable expectations for fitting daily treatments around a trial study and the considerations of the impact on family life and other commitments.

At a clinic appointment I attended when I was about 16, the day before a careers fair, I recall the doctor telling me not to worry about a career and just to enjoy life. I'm pleased to say that I've reached my forties and have been able to both enjoy life and have a fulfilling career, as well as the joy of starting a family. I hope that I'll be around to see my son grow up and follow his dreams in life – he currently wants to be an artist (or a superhero!). With continuing advances in treatments, brought about through clinical trials, that reality is becoming more and more the norm.

Martin, (age 43)

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What about trials involving babies and children?

Clinical trials are not just for adults. Cystic fibrosis, as you know, affects children from birth and treatment is vital to maintain good health and slow the deterioration. Clinical trials therefore often need to include children or even babies. Children are not just small adults and may react differently to the same medication; it's not sufficient or effective to simply adapt the dose.

As with clinical trials involving adults, trials involving children are very carefully regulated, and approval to include children will only be given if the potential benefits can be shown to outweigh any possible risks.

Obviously, babies and younger children are not able to give informed consent, and special consideration is given to the fact that children are less likely than adults (or completely unable) to be able to communicate their experiences of participating in a trial. Talk to your healthcare professional or CF research team about the consent process for including children and babies in research or find out more on our website by visiting cysticfibrosis.org.uk/takingpart.

Some parents can struggle with the decision of whether to enrol their child in a clinical trial, and it is understandable to have concerns.

Talking to your child/children openly about research and trials from an early age can make it easier to reach decisions about taking part in research when opportunities arise.

"Both of my children took part in a clinical trial looking at the effects of Tobramycin on the kidneys. The team approached me about it and gave me lots of information, and I felt happy that I knew enough to agree to enrol my children. The research team always had time for questions, which made me more comfortable. I would be happy to consider further trials in future, but would need to be fully informed about the trial and any potential risks."

Lisa, mum to two children with cystic fibrosis, aged 4 and 8

"Our daughter is only three, so we haven't thought too much about participation in research yet, but we're interested to know what trials are happening."

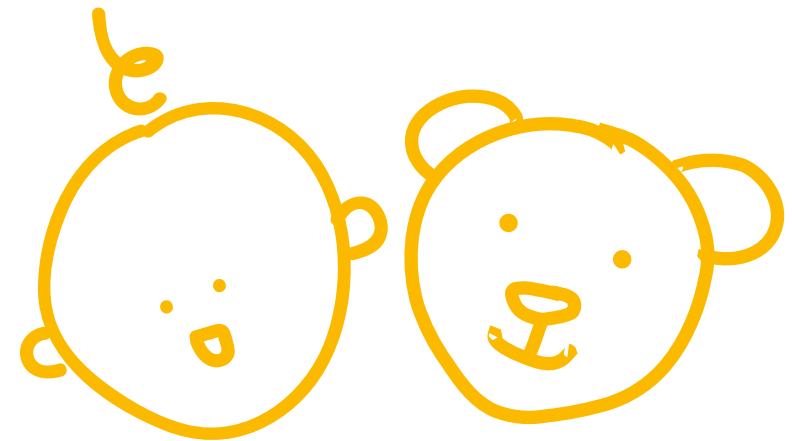
Paul, dad to 3-year-old with cystic fibrosis

"My son hasn't taken part in any trials yet, but I would definitely consider it if asked. It could potentially help him and other children with cystic fibrosis."

Vikki, mum to 15-month-old with cystic fibrosis

"It's important that people can see the condition isn't being ignored, and that there is research happening."

Yasmin, mum to a 15-year-old with cystic fibrosis





What will it mean
for me?





"Taking part in trials is a chance to learn more about yourself and your condition."

Martin, (age 43)

What will taking part mean for me?

Clinical trials take many different forms, and what is required will vary greatly depending on what is being researched.

Many trials involve additional 'research visits' to your CF centre (or the CF centre running the trial), where questionnaires about how you've been feeling will be completed and any necessary clinical measures taken i.e blood test and spirometry. Researchers will also look out for any side effects and ask about any positive or negative changes you have experienced.

You will be monitored regularly during the trial and for follow up appointments. Sometimes this means going to your CF centre more often than you would normally.

There is always the risk that the trial treatment could result in some unwanted side effects. However, the extra attention you receive during clinical trial participation means that any changes in your health, whether related to CF or to the treatment you are having, is likely to be picked up sooner than if you weren't in a trial.

Some people with CF have said that they found the extra focus on their health made them think more about their condition, which could be a good or bad thing.

"A big plus for me is that as well as helping further research, there is also the possibility that you will receive some beneficial effects on your health."

Cleo, (age 32)

"I can only speak for myself but it has been very rewarding to give something back to the community. Even if I hadn't noticed any effects, the fact that my involvement was potentially helping thousands of other people with CF was quite reassuring."

Simon, (age 35)



“Taking part in a trial brings feeling of extra love and attention – more time taken with extra measurements and statistics.”

Adam, (age 38, adult with cystic fibrosis)

“Generally it has been a very positive experience. I found it quite rewarding to be involved with something that could benefit people with cystic fibrosis. Health-wise it has also worked very well. I was on IVs every three to four months. Now it’s every year and a half.”

Simon, (age 35)

“For me, taking part in trials has been life-changing - my lung function improved dramatically, I could tell I was receiving the new treatment.”

Jane, (age 53)

How long do clinical trials last?

Although trials can run for many months, or even years, it doesn't mean that you would be expected to participate for this duration! There are several reasons why trials can be lengthy:

- It can take a long time to recruit enough people to take part in the trial (even a small number of people with a rare mutation)
- It may involve giving a treatment over a long period of time
- It may be important to follow up with patients over a long period of time to get a reliable picture of the long-term effects of a treatment

Understandably, for those who stand to benefit from a new treatment in trials, and particularly for those with life-shortening conditions, the length of the trial process can be frustrating.

Speak to the trial team to get a full picture of how long you would need to take part, and how often you will need to visit the CF centre for research visits.

If a trial is being conducted at your own CF centre, appointments may be scheduled to take place before or after your routine clinic appointments, to try and minimise the number of visits you have to make. However, this isn't always possible, and while trials generally aim to be flexible, some appointments may be needed during school or work hours. Inevitably, trial participation can sometimes present challenges in juggling the trial with your personal life, employment, education or other commitments.

“Reading about things that are years off can be hard mentally.”

David, (age 33)



Are clinical trials safe?

"Time was probably the biggest question I had – I hadn't approached work beforehand and I wanted to get as much info as possible before I talked to my boss. I ended up having a fair amount of time out of work, attending

hospital once a month, sometimes for the whole day. I knew what would be involved beforehand and luckily my employers were very understanding."

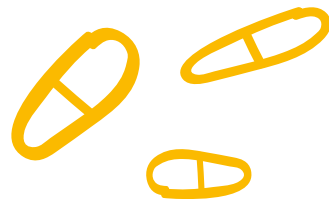
Simon, (age 35)

"I was at work, and my health wasn't fantastic. Juggling the trial with work was a challenge, but trial appointments would normally coincide with my routine clinic appointments which helped lessen the burden."

Jamie, (age 32, adult with cystic fibrosis)

"I was working as a teacher at the time – but was able to do the trial in school holidays over eight weeks in the summer."

Cleo, (age 32)



Understandably, safety is often a key concern people have about clinical trials. Clinical trials are carefully controlled, regulated and organised to minimise risk to participants. Serious side effects are very rare and clinical trials are now safer than ever.

New medications will have been checked using human and animal cells, before ever being administered to humans. However, because some clinical trial interventions are investigational, there is always a small possibility of unpleasant or potentially serious side effects of any new treatment. It's worth remembering that not all trials are for new medications – some trials involve commonly prescribed medications to see if treatments effective for other conditions are also effective in cystic fibrosis.

"Before taking part, you do think will it make me feel better or worse?"

Jane, (age 53)

During the informed consent process, you should be told everything that the researchers know about any possible risks and side effects so that you can make an informed choice about whether to participate.

Every trial has an independent Data Safety Monitoring Board (DSMB), and if anything happens during a trial, such as a bad reaction, it is immediately reported to the DSMB. The board will stop a trial if there is any doubt about the safety of the treatment.



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When I was younger I was involved in various studies: Azithromycin study, I-neb nebuliser study, Creon 10,000 study and more recently the Gene Therapy Study as well as various smaller clinical studies which involved providing lung function, sputum and blood samples.

I know that safety studies have been conducted before a trial takes place, so I've never felt there's too much to be concerned about. However, there were times when I hadn't taken on board what would be involved; having done one trial, I assumed others would follow suit, but there are differences.

Even signing a consent form, you aren't obliged to participate. Clinical trials won't affect your care either.

Taking part in clinical trials has really helped improve my understanding of my condition. For example, with the gene therapy trial I was given a peak flow meter, which I had to do every week and record my results. Due to this closer monitoring, I was more aware of when I was having a dip in my health, and could let my team know. Trials have also given me access to newer treatments, which have benefited my health. Another plus for me is the sense of feeling useful, and helping the wider community of people with cystic fibrosis.

Jamie, (age 32)



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Will my information be kept confidential?

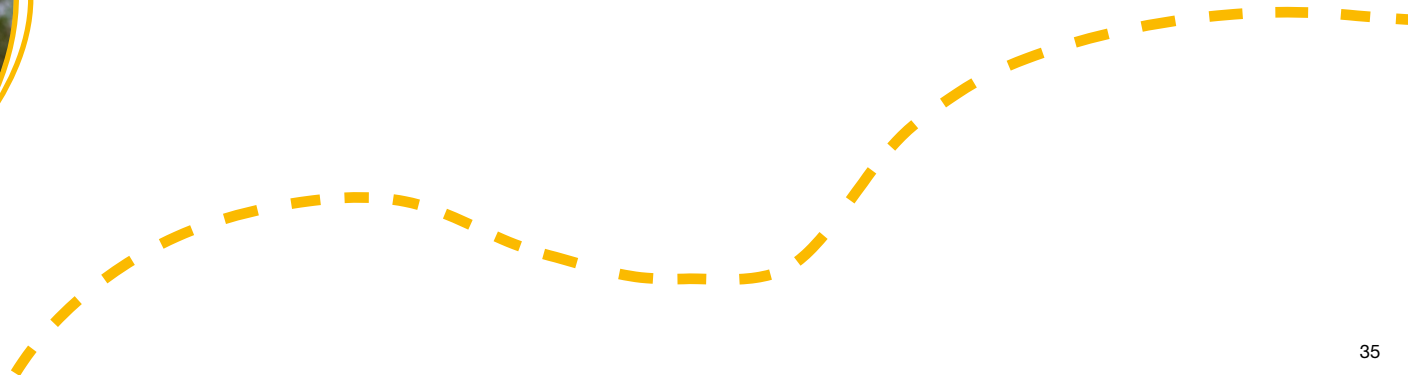
Yes. If you agree to take part in a clinical trial, any information that is collected about you will be kept confidential. The researchers cannot tell anyone that you are taking part in the trial without asking you first.

Once the trial has finished, the results may be published in a scientific journal, or at a conference, but no identifying information about participants in the trial would be shared.

Withdrawing from a clinical trial

You can withdraw from the trial at any time, and without giving a reason. This will not impact on your care in any way.

Depending on the type of trial, the participant may be given advice on how to withdraw from the trial safely, for example when new medicines are being investigated.

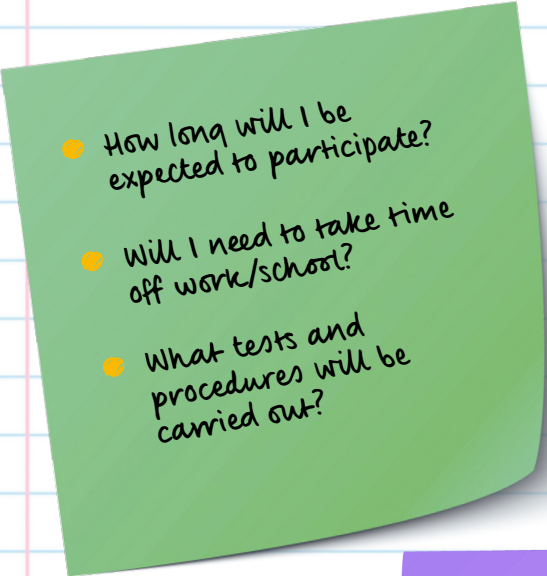


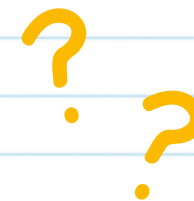
Questions worth considering before starting a clinical trial

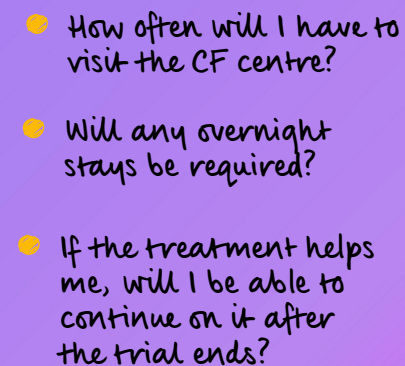
There's a lot to take in about clinical trials and some of the process and language might be new to you. As mentioned throughout this booklet, it's really important you have all the facts you need to make an informed decision about whether to participate in a clinical trial.

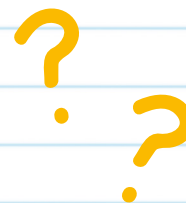
People with CF have helped us put together a checklist of questions you may wish to ask to help you reach a decision. There is space to make notes at the end of this booklet.

- What are the aims of the trial and how will it help people with CF?
- Will I be told if I will receive the treatment or a control/placebo?
- What kind of trial is it?
- What are the potential risks/benefits?
- What is the treatment being studied?
- Will I be paid to take part?
- Has the treatment been given to people with CF before and, if so, what were the results?
- What kind of follow up will there be?
- Will some of the people on the trial be given a placebo?
- How will I be notified of the results?
- Who should I speak to if I have a question or concern?

- 
- How long will I be expected to participate?
 - Will I need to take time off work/school?
 - What tests and procedures will be carried out?



- 
- How often will I have to visit the CF centre?
 - Will any overnight stays be required?
 - If the treatment helps me, will I be able to continue on it after the trial ends?





After taking part





After taking part

Depending on what stage of the trial you joined, it is likely that the trial will continue even when you have stopped participating. Most participants want to know

what the outcome of the trial has been - just keep in mind that it could be a significant length of time after your participation has ended before results are available.

How can I track progress of a clinical trial?

At the end of the trial, a summary of the results should be made available to everyone who took part and will also be published so that others can use the information to help them make decisions about treatment and healthcare – this will be in a scientific journal, and possibly at a conference too.

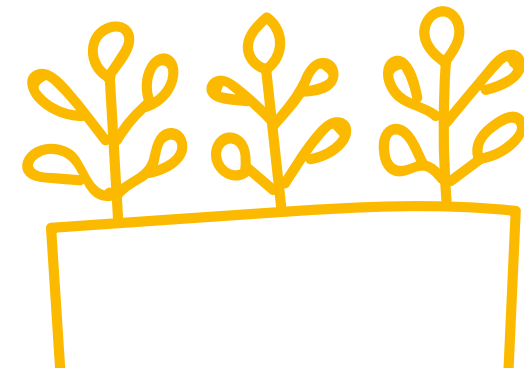
to proceed, the trial has still been worthwhile. Without conducting trials, questions about safety and efficacy of potential new treatments would go unanswered, and potentially valuable treatments would never reach patients.

Where a trial doesn't have the predicted outcome, for example if the intervention is proved to be ineffective, or not effective enough

All trial results are building blocks in our search for the best treatment and care so a disappointing outcome in one trial is still a successful study!

“For me one of the main downsides is that even if you found out the treatment was working for you, there is no guarantee you would have access to it after the trial. I'd worry about losing access to an ineffective treatment.”

Simon, (age 35)



Stop the Clock on cystic fibrosis

Will I be eligible to stay on the treatment?

Possibly, but there maybe a gap between when the trial finishes and when you can continue to take the treatment.

Where a treatment has been shown to be effective, research teams, pharmaceutical companies and patient organisations will all be keen to see the treatment made available as quickly as possible.

However, even once a treatment has been shown to be effective

there can be a significant wait as the NHS, pharmaceutical companies and other organisations negotiate on the price of the treatment.

In this case you will continue to be given your standard treatment.

Doctors may be able to prescribe the treatment to certain patients via an 'expanded access programme' (also known as a 'patient access scheme' (PAS) or 'compassionate use' scheme).

Did you know?

In some situations, while negotiations are underway to bring a new treatment to patients, doctors may be able to prescribe the treatment to certain patients via an 'expanded access programme' (also known as a 'compassionate use scheme'), in which the pharmaceutical company makes the medicine available on a case by case basis.

Such programmes usually involve strict eligibility criteria, and the doctor prescribing the medicine would have to take full responsibility for prescribing the treatment for their patient.

In an 'expanded access programme', patients are usually followed up in the same way as if they were on a clinical trial.

Because the medicines in question are unlicensed, and therefore there may not be detailed information available on their safety and efficacy, they are usually limited to patients for whom conventional treatment options are no longer effective and who are often very unwell.

REMEMBER Clinical trials cannot happen without us - people living with cystic fibrosis - choosing to take part.

Oli Rayner, (age 42, adult with cystic fibrosis)

Clinical trials are essential in the development of new and effective treatments for cystic fibrosis; the significant progresses in treatment and care over recent decades are directly attributable to the research successes, which are dependent on clinical trials. Participation in a trial is a very personal decision and needs consideration. We hope that this booklet has helped to give you the information you need or

pointed you in the right direction to find further details.

We are very grateful to everyone who helped develop this booklet. To the volunteers who shared their stories, the experts who contributed their time and expertise and to the Cystic Fibrosis Foundation (USA) who generously supported us financially

- thank you!



Finding out more

The Clinical Trials Accelerator Platform

The Clinical Trials Accelerator Platform (Trials Accelerator) is a ground-breaking UK-wide initiative, developed by the Cystic Fibrosis Trust, to increase access to clinical trial opportunities for people living with cystic fibrosis. We are supporting CF centres to increase the speed at which clinical trials are set-up and delivered, which all helps to encourage the timeliest delivery of new, effective treatments.

Through the Trials Accelerator, we are building a network of CF centres that will lead in the delivery of high-impact clinical trials.

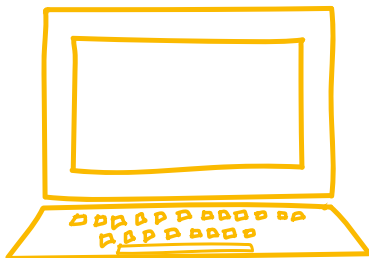
In addition to this, as mentioned earlier in this booklet, we have also created an online Clinical Trials Digital Hub, which provides information about taking part in clinical trials, and through the CF Trials Tracker, lists opportunities to participate in CF clinical trials. You can access the Clinical Trials Digital Hub and the CF Trials Tracker at cysticfibrosis.org.uk/clinicaltrials.

You can visit cysticfibrosis.org.uk/trialsaccelerator for the latest updates on the Trials Accelerator, including participating centres.

Get Involved

Patient & Public Involvement (PPI) is integral to the Trials Accelerator and differs from participation in a trial. If you want to get involved and have your say in how research for CF is conducted visit cysticfibrosis.org.uk/PPI.

To register your interest in getting involved or to receive news updates, email clinicaltrials@cysticfibrosis.org.uk



Cystic Fibrosis Trust

The Cystic Fibrosis Trust website contains lots of information, resources and videos about clinical trials www.cysticfibrosis.org.uk/clinicaltrials

If you wish to discuss clinical trials with other people with CF, parents and carers, who have previously participated in a trial, we have **Peer Advocates** who are happy to share their experiences.



Please contact

clinicaltrials@cysticfibrosis.org.uk,
or join our forums
www.cysticfibrosis.org.uk/forums.

Useful websites

Generation R

generationr.org.uk is an organisation dedicated to involving young people in the design and delivery of research.

Health Talk www.healthtalk.org has a section dedicated to people's experiences of clinical trials.

For guidelines on research involving children www.mrc.ac.uk/documents/pdf/medical-research-involving-children/





Glossary

The following are some common terms you may hear relating to clinical trials.

Adverse event: a medical occurrence which may or may not have been caused by a trial medication.

Adverse reaction: an unfavourable and unintended side-effect experienced by a clinical trial participant which is directly related to the treatment being administered.

Blind trial: a clinical trial in which the participants do not know whether they are receiving the intervention being tested or a control treatment/placebo. See also double blind trial.

Chief Investigator: the lead researcher/clinician who has overall responsibility for a clinical trial.

Comparative / controlled trial: a trial in which an investigational treatment is compared to an existing treatment.

Compassionate use scheme: a treatment option that allows medicines to be accessed by seriously ill patients, before they have been officially approved for use (also known as an 'expanded access programme').

Control group: the group of participants in a clinical trial that receives the control treatment (placebo or standard treatment), rather than the investigational treatment.

Data: clinical information about the safety and effectiveness of treatments in a clinical trial.

Double blind trial: a clinical trial in which neither the participant nor the clinical/research team knows who is receiving the trial medication and who is receiving the existing treatment or a placebo.

Efficacy: the effectiveness of a treatment in achieving its intended purpose.

Eligibility criteria: the inclusion and exclusion criteria which determines who can take part in the trial. These criteria are based on factors such as age, CF genotype, previous treatment history, and existence of other medical conditions.

Ethics: ethics are a critical aspect of clinical trials, and a key concern for research involving humans and animals. Before a trial is given the necessary regulatory approval to open, regulatory bodies will seek to ensure that the trial is conducted by qualified researchers and has a valid purpose, that participants will be fully informed as to what the trial will involve, and that the trial will not cause undue harm to participants.

Homozygous: where someone has two copies of the same CF gene – for example two copies of dF508.

Heterozygous: where someone has two variations of the CF gene – for example, one copy of dF508 and one other variation such as G551D or R117H.

Intervention: a treatment (medication or other) being investigated in a clinical trial.

Length of participation: the length of time a participant will take part in a trial, from the first to the last appointment.

Observational study: a study where there is no intervention, but certain healthcare measures are observed and recorded over a period of time.

Open-label trial: unlike a blind trial, a clinical trial in which the researchers and the trial participants know they are taking the trial medication.

Open-label extension: normally occurs after a double blind trial; participants are invited to enrol on an extension study which will involve taking the trial medication for a period of time. No placebos are used in extension studies and both participant and researcher know the participant is taking the trial medication.

Outcome measures: the measurements or other assessments used to determine whether the trial medication is safe and effective.

Placebo: a dummy medication, used to compare against a trial medication. It has no medicinal effect.

Phase: the different stages involved in the development of a new medication. Phase 1 focuses on initial safety in people. Phase 2 evaluates safety, correct dose and early signs of whether the medication works. Phase 3 is the stage before medication licensing and looks at safety and medication efficacy. Phase 4 evaluates longer term use of a medication after it has been licensed.

Primary outcomes: answers the most important questions about the trial i.e. is the trial medication safe and effective?

Principal Investigator: the researcher (clinician) who is responsible for a clinical trial at a particular trial site.

Protocol: a scientific document which outlines the design of a clinical trial and how it will be conducted.

Randomised controlled trial (RCT): a trial in which participants are randomly assigned to one of two or more treatment arms of a clinical trial (see 'Treatment arm'). This is usually done by computer, so that each group has a similar mix of people of different ages, sexes and states of health.

Recruitment target: the number of participants who need to be recruited for the trial in the UK.

Sample size: the number of participants in a clinical trial.

Screening: the first official assessment for a participant in a trial, where it is checked if the participant meets all of the inclusion and exclusion criteria.

Secondary outcomes: answers other questions about the trial e.g. time to next pulmonary exacerbation.

Sponsor: the individual, company, institution or organisation responsible for initiation, management, safety monitoring and financing of a clinical trial.

Therapeutic category: the type of treatment or therapy being studied. A therapy could range from a medication addressing a particular characteristic of CF to a device or activity i.e. exercise.

Treatment arm: the group a participant is assigned to which determines the trial treatment regime they will receive, i.e. the trial medication or placebo.

Trial site: the CF centre or other establishment at which the clinical trial takes place – multi-site trials are conducted at more than one CF centre.

Your Notes



Find us online

cysticfibrosis.org.uk
facebook.com/cftrust
twitter.com/cftrust
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instagram/cftrustuk

