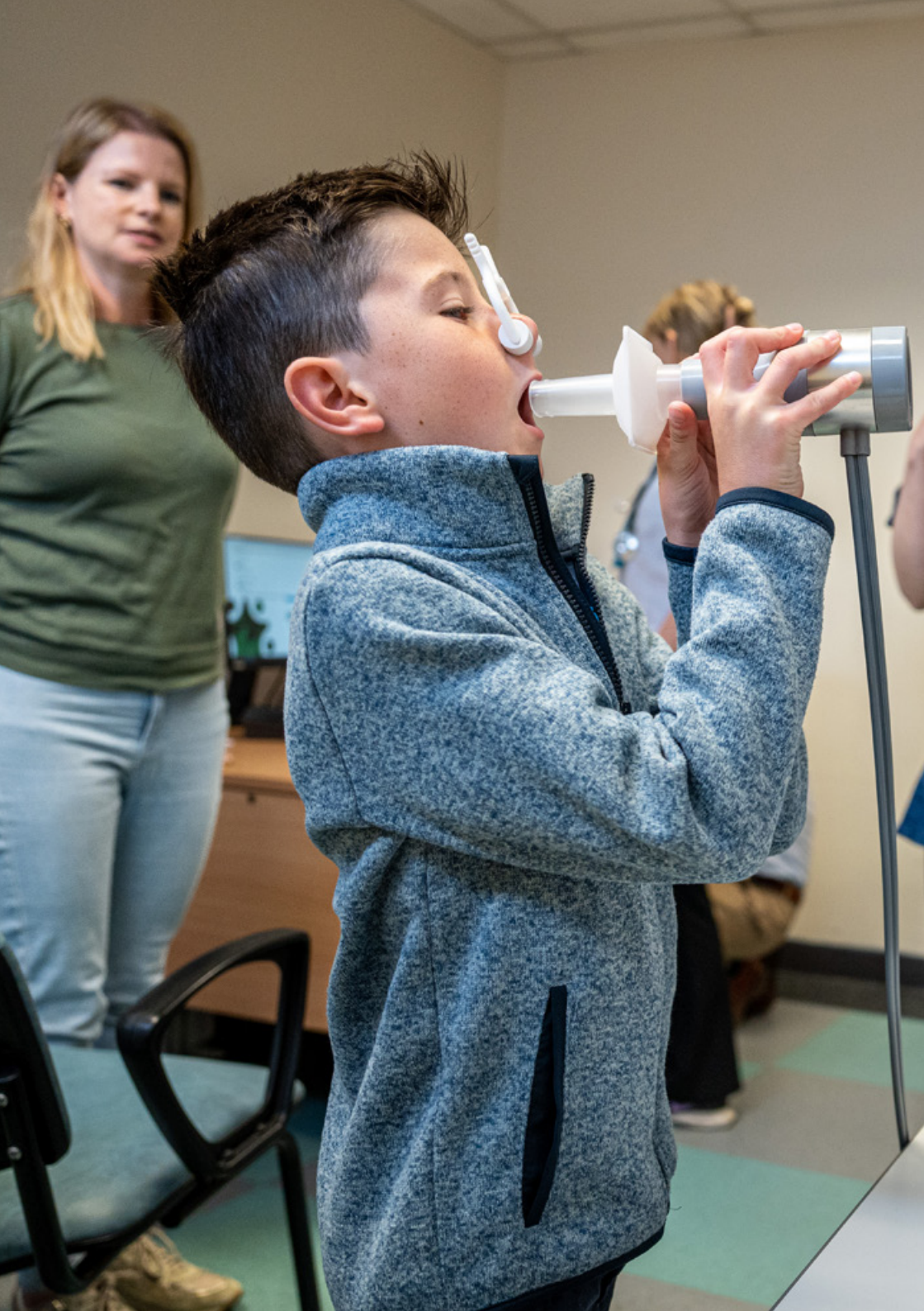




An introduction to taking part in **clinical trials**

Find out more about what clinical trials are,
why they're important and what's involved



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Introduction

There are lots of ways to be involved in cystic fibrosis (CF) research. Taking part in a clinical trial is just one of them.

Clinical trials aim to improve treatment and care for people with CF. All of the current medicines and treatments we have for CF are thanks to people supporting and taking part in clinical trials.

Taking part in a trial may mean you are one of the first people to benefit from a new treatment or intervention, and some people find that extra

monitoring can help them understand their CF better. However, it should always be a well thought out decision.

We hope the information in this booklet helps you understand more about how trials work, what happens during a trial, who can take part, and how to find current trials.

You can find lots more information, news and personal stories about clinical trials on the Clinical Trials Hub at cysticfibrosis.org.uk/clinicaltrials

What is the Clinical Trials Accelerator Platform?

Cystic Fibrosis Trust launched the Clinical Trials Accelerator Platform (CTAP) in 2017 to support CF clinical trials in the UK. By bringing together a network of CTAP centres and funding a team of CF trial coordinators to run clinical trials, we are accelerating the development of new treatments and helping more people take part in research. Since CTAP was formed, more than 4,000 people in the UK have taken part in trials.





I only have positive things to say about being part of trials. It feels good to take control, and taking part in a trial gave me the opportunity to be proactive. If we want better treatments, we need to participate in that process.”

Stuart, who has CF

What are clinical trials?

A clinical trial is a type of research study. They investigate the effect of a new type of treatment on human health. A treatment can be:

- a new medicine
- an existing medicine used in a new way
- stopping an existing treatment
- a medical device such as a nebuliser or app
- behavioural or lifestyle changes, such as modifying diet or physical activity.

Clinical trials are the only way we currently have to assess whether an intervention works and is safe. They are an essential step before a new intervention is licensed and available as a prescription.



Why should I be interested in clinical trials?

Every medicine you and everyone you know has ever taken, even over-the-counter ones like paracetamol, will have gone through a clinical trial at some point. They matter to everyone.

The trials taking place today will continue to improve the lives of everyone with CF. While we have seen huge developments in CF treatment over the last few decades, there is still a lot of progress to be made. This is particularly true for people who aren't currently able to benefit from modulator treatments.

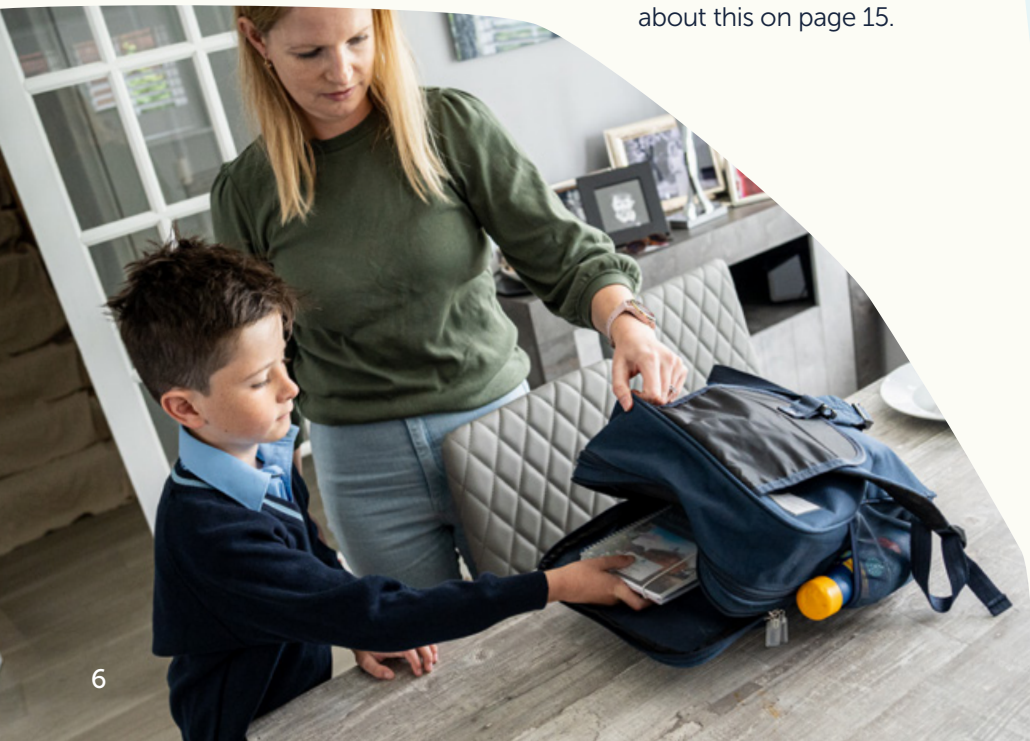
Over the next few years, we hope to see trials in genetic therapies, which

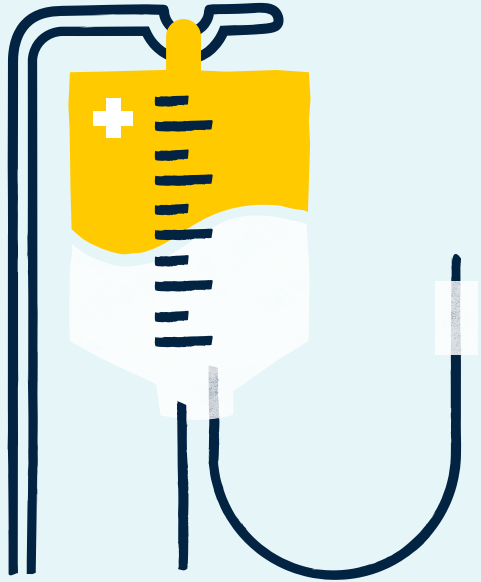
may be able to treat the underlying cause of CF. There will also be a focus on finding new and more effective ways to treat the symptoms of CF.

Other trials are focusing on improving the quality of life of people with CF; for example, through better anti-infection care or improving digestive symptoms.

For these developments to be possible, we need people with CF to support clinical trials.

Taking part in a trial is one way to be involved. You can also join our Involvement Group to help shape future trials by sharing your experience of CF. Find out more about this on page 15.





How do clinical trials work?

Clinical trials are set up and run by pharmaceutical companies, clinical researchers at hospitals or universities, doctors, and other healthcare professionals. Sometimes, we call the people who run trials ‘sponsors’.

All clinical trials in the UK are tightly regulated to make sure they are safe, ethical and well organised.

Types and phases of trial

You might come across terms for different types of clinical trials. The type of trial depends on what the study aims to find out.

Most clinical trials for new treatments are ‘controlled trials’. This is where the new treatment is compared to a ‘control’, which can be a standard treatment or no treatment (‘placebo’).

Clinical trials for new treatments go through several different steps, or ‘phases’, before a treatment can be approved for use. Earlier phase trials look at the safety and side effects of a new treatment. Later phases look at whether it is better than current treatments.

Some research studies may simply be ‘observational’. They look at different health measures, such as mood or FEV1, over a period of time. This is sometimes called real-world evidence (RWE).

You can find more information about trial types and phases on the Clinical Trials Hub at cysticfibrosis.org.uk/clinicaltrials

Can I take part in a clinical trial?

All clinical trials will specify what requirements you must meet to take part in a particular trial. This is referred to as the 'eligibility criteria'.

These can include:



- your age



- your CF genotype



- your FEV1 status (lung function)



- whether you are pregnant or planning to start a family



- your current treatments



- what other medical conditions you may have.

Sometimes, you and your CF team can easily tell if you can take part in a trial. At other times, you may not know until you go for a screening visit after you've consented to take part in the trial. This will usually be at the hospital or CF centre where the trial is taking place. This may or may not be your usual CF care centre. Your health and medical history will be checked against the trial's eligibility criteria.

Sometimes, people are told they cannot take part in the trial. This can be for a variety of reasons, such as your FEV1 being too high or too low. Being told you cannot take part in a clinical trial can understandably be very disappointing. It's important that clinical trials have strict criteria in place to make sure the trial is as safe and accurate as possible.

Even if you don't meet the criteria at the start of a trial, it might not necessarily exclude you from taking part in the trial at a later date or from taking part in a different trial. Your health might change, or other trials will have different criteria.

Everyone with CF, whatever their background, should be able to participate in the clinical trials that they're eligible for. It is important that clinical trials have a diverse representation of participants that reflect the entire CF community. This is because our understanding of how effective and safe a new treatment is comes from the people who take part in clinical trials.

If you would like support to take part in a trial, this is available. Please email clinicaltrials@cysticfibrosis.org.uk or contact the trial coordinator to discuss this.

Should I take part in a clinical trial?

Deciding whether or not to take part in a clinical trial is a personal decision. You should never feel pressured to take part. If you do join a trial, you can withdraw at any time.

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

You may also find it helpful to read more stories and advice from people who have taken part in trials on the Clinical Trials Hub at [cysticfibrosis.org.uk/clinicaltrials](https://www.cysticfibrosis.org.uk/clinicaltrials)

“My son Joshua is eight years old. Although we haven’t been offered a clinical trial yet, Joshua has some understanding of trials. He is inspired by science; he likes to know how drugs work in the body, and he is someone who likes to help others. He would like to take part in the right trial, not just for his own benefit but for the greater good of everyone with CF. The main thing we would consider is whether he would need to come off Kaftrio to take part. Other considerations would be what sort of procedures will be involved and time out of school. Sometimes it comes down to timing, and saying no isn’t the wrong thing if that is the right decision for your family.”

Esther, mum to Joshua, who has CF



What could I be asked to do in a clinical trial?

What you'll be asked to do will vary depending on what is being researched. There are many different treatments that are being developed through clinical trials, including:



- antibiotics and other treatments for infections



- new modulators



- genetic therapies



- anti-inflammatory medicines.

Alternatively, you could be asked to change your lifestyle as part of a trial, such as the way you do your airway clearance to clear excess mucus from your lungs (physio). You could be asked to trial a piece of medical equipment that hasn't been used before in CF, like an insulin pump. Or trial the best way to treat a lung infection using existing medicines.

Some trials have in-person visits, but others may be partially or

fully virtual, with telephone calls or remote monitoring devices like smartwatches or apps.

There are many different ways that a trial team can measure the effect of a treatment. Examples of things a trial team might measure or test include:

- lung function, using spirometry to measure FEV1, or a chest scan
- a blood sample
- a urine sample
- a sputum sample
- an oropharyngeal or nasal swab
- sweat test
- vital signs, such as using blood pressure or electrocardiogram (ECG).

You might also be asked to complete a questionnaire or interview about your views and experiences.

I get a bit emotional when I think about the NHS and my CF team. They've helped me out so much, and I want to contribute to the future of CF. The way I see it, if a drug works for me, it might work for someone else and, if I can help someone at a young age to live a more normal life, then I would do that."

Guv, who has CF



Want to get involved
in a clinical trial?



Talk to your CF
care centre



Contact a CF trial
coordinator



Visit the
Trials Tracker



Contact the CTAP
team at the Trust



How can I find clinical trials?

We have lots of information on the Clinical Trials Hub about how clinical trials work and what's involved in taking part in a trial. There, you can also find the Trials Tracker, where you can find CF trials that are currently open in the UK and get in touch with the coordinator for each trial.

Other ways to find current trials

- Talk to your CF team. They can tell you about opportunities in your area and further afield.
- Contact a CF trial coordinator. You can find a list of all CTAP trial coordinators on the Clinical Trials Hub. For a particular trial, see the Trials Tracker listing.
- Get in touch with CTAP. Email **clinicaltrials@cysticfibrosis.org.uk** if you have questions about current or upcoming trials.

A trial may run at several CF centres across the UK, Europe, or worldwide, or it might just be taking place at one CF centre only.

If there are no trials near you, try widening your search area or contact us at **clinicaltrials@cysticfibrosis.org.uk**. You may have the option to travel to a different CF centre to take part in a trial. Some trials may be able to reimburse you for travel and accommodation costs if you have to travel a long way.

To find out more about the latest trials and Involvement opportunities, and to sign up to our Clinical Trials and Involvement newsletter, visit the Clinical Trials Hub at **cysticfibrosis.org.uk/clinicaltrials**

Are clinical trials safe?

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 given to humans. However, there is always a small possibility of unpleasant or potentially serious side effects of any new treatment. You can stop taking part in a trial at any point, without needing to give a reason. This will not affect your CF treatment.

You will be monitored closely throughout any trial and afterwards for the follow-up period. Trial teams will look out for any side effects and ask about any positive or negative changes you experience. The extra attention to your health during a clinical trial means that any changes, whether related to CF or to treatment, are likely to be picked up sooner than if you weren't in a trial.

Every trial also 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.

I I didn't find the trial particularly onerous even though we had regular meetings. My appointments were in the research centre, so you don't have to deal with being in the hospital. I had a really good employer who realised that it was best for me to take some time to get better, and it all worked well."

Stuart, who has CF



Before you take part in a clinical trial

The trial team are responsible for making sure that you are fully informed about the trial at all times. They must make sure that you have a full understanding of what the trial is, what it involves and any potential benefits or risks they are aware of. This is called informed consent.

Informed consent is a process that begins when you are first told about a trial and continues throughout your participation. You can find out more about this on the Clinical Trials Hub: cysticfibrosis.org.uk/clinicaltrials

It is important to know that you might not take a new treatment by participating in a trial. You may take a standard treatment, which you might be taking already, or a placebo. This still contributes to the trial by helping identify the best treatment for yourself and others.

People with CF have helped us put together a list of questions you may wish to ask your CF team or trial coordinator to help you reach a decision.

1. What are the potential risks and benefits?
2. Has the treatment been given to people with CF before, and if so, what were the results?
3. Will some of the people on the trial be given a placebo or a standard treatment? Will I be told if I am in the treatment group or the control group?
4. Will my expenses, like travel and refreshments, be paid?
5. How long is the trial? How many visits will there be, and are any virtual? Will I have to take time off work or school?
6. Who should I speak to if I have a question or concern?
7. How long will I be expected to participate? What kind of follow-up will there be?
8. If the treatment helps me, will I be able to continue on it after the trial ends?
9. Who will tell me what the results of the trial are and how long after the trial has ended?





I don't like to be away from my son, so the number of days I'd be required to take part in a trial is always a deciding factor for me. When considering a trial, I also think about what else might be coming down the road which might be better suited to me or is likely to be more successful. I ask my team about any trial results either from earlier phases or from other countries to help inform my decision."

Guv, who has CF

Can children and babies take part in clinical trials?

Since children and babies also take medicines, clinical trials sometimes need to include them. Trials involving children are very carefully regulated. They are only approved if the potential benefits can be shown to outweigh any possible risks.

Babies and children under 16 years cannot give informed consent and will need an appropriate person to consent for them. Children and young

people should be involved in the decision-making whenever possible.

Some parents can struggle with the decision of whether to enrol their child in a clinical trial, and it is understandable to have concerns. You can find more information about clinical trials for young people and talking to your child about trials on the Clinical Trials Hub: [cysticfibrosis.org.uk/youngpeopletrials](https://www.cysticfibrosis.org.uk/youngpeopletrials)



Help shape future clinical trials

Our Involvement Group helps ensure that the experiences and preferences of people with CF are taken into account when clinical trials are designed. As part of the work we do through CTAP, we help to make sure people with CF are involved at various

stages of trial design so researchers can understand how to adapt trials to better meet their needs and reduce the burden of taking part.

To find out more, visit [cysticfibrosis.org.uk/getinvolvedwithtrials](https://www.cysticfibrosis.org.uk/getinvolvedwithtrials)



I have enjoyed being a member of Cystic Fibrosis Trust's Involvement Group. I have participated in a number of research focus groups and have seen that this can impact people living with CF in a positive way. My main focus is, and always has been, to help the younger generation in some way.

"Advances in treatments, and perhaps safely stopping some medications, means that adults and children with CF can live longer and healthier lives. I know the questions I have about my personal CF can only be answered through research and clinical trials. Those trials can only give the answers if we all pull together."

Tonia, who has CF





The advances in CF treatments over the last two decades have been phenomenal, well beyond anything that was predicted when I started specialising in CF.

“These improvements have all been driven by careful and rigorous clinical trials and could not have been delivered without the support of thousands of people with CF and their families. We are also proud of the role that CTAP has played in this by supporting trials and ensuring they are delivered to as many centres as possible across the UK.

“Yet despite all the progress, research and trials remain important in CF. For those without access to modulators, there are huge

advances still to be made. CTAP is supporting trials in genetic therapies, and in addition to this, we are still looking for better modulator therapies, better ways of treating infection in CF, and better ways of managing other aspects of CF like diabetes or gut symptoms.

“For these trials to succeed, though, we still need the support and involvement of people with CF and their families, without whom none of this would be possible. It’s a journey that Cystic Fibrosis Trust will be leading on, and we hope you can join us.”

**Professor Alex Horsley,
CF Adult Consultant and
Senior Leader for CTAP**



Further information

Find more information resources about living with cystic fibrosis at cysticfibrosis.org.uk/information

Our Helpline is open 10am–4pm Monday to Friday. It's available to anyone looking for information or support with any part of cystic fibrosis, a listening ear, or just to talk things through.


How to reach us

- Call **0300 373 1000** or **020 3795 2184**
- Email helpline@cysticfibrosis.org.uk
- Chat with us on Facebook, Twitter or Instagram
- Message us on WhatsApp on **07361 582053**

Visit cysticfibrosis.org.uk/helpline for more information.

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A man with a beard, wearing a light-colored jacket and dark trousers, is sitting on a balcony with a wooden floor. He is leaning against a dark metal railing and looking out over a harbor. In the background, there is a large white boat with a blue crane-like structure. The sky is blue with some clouds. The overall scene is peaceful and contemplative.

I know from focus groups I have taken part in that many of the trials in the pipeline are for people with rare mutations and are likely to be running at just a few specialist centres. I wouldn't mind travelling to another centre to take part in a trial as long as my travel expenses were covered."

Guv, who has CF

Cystic Fibrosis Trust

Cystic Fibrosis Trust is the charity uniting people to stop cystic fibrosis. Our community will improve care, speak out, support each other and fund vital research as we race towards effective treatments for all.

We won't stop until everyone can live without the limits of cystic fibrosis.

The information included in this publication is not intended to replace any advice you may receive from your doctor or CF multidisciplinary team and it is important that you seek medical advice whenever considering a change of treatment.

We welcome your feedback on our resources.

You can also ask for this resource in large print or as a text file. Email infoteam@cysticfibrosis.org.uk

cysticfibrosis.org.uk

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