Cystic Fibrosis Trust

Uniting for a life unlimited

Our research goals

Accelerating progress towards a life unlimited

January 2025

Contents

Summary

Cystic Fibrosis Trust is the charity uniting people to stop cystic fibrosis (CF). We fund vital research, improve care, speak out and race towards effective treatments for all. Cystic Fibrosis Trust is here to make sure everyone with cystic fibrosis can live without limits.

Since 1964, we've supported people with cystic fibrosis to live longer, healthier lives – and we won't stop until everyone can live without limits imposed by CF.

Through our research goals we will accelerate progress towards a future where everyone with CF can live a life unlimited.

We will develop new and improved treatments for everyone, find better ways to diagnose and treat lung infections, treat all CF symptoms throughout the body and enable people with CF to live longer, healthier lives.

These goals are informed by the research priorities of the CF community. To achieve them we will fund world class research, build effective partnerships, provide internationally recognised infrastructure and harness high quality healthcare data. People with CF will be involved in contributing to and shaping all of these activities every step of the way.

Goal: To develop effective treatments for all

We will develop new and better treatments that work for everyone with cystic fibrosis, and stop lives being limited by the condition.

Goal: To improve the diagnosis and treatment of CF lung infections and maintain lung health

We will find better ways to diagnose and treat lung infections, tackle antimicrobial resistance, and stop CF disrupting lives.

Goal: To treat all of the symptoms of CF throughout the body

We will improve understanding of the wide range of symptoms and complications of CF happening throughout the body and make progress in treating them effectively.

Goal: To enable people with CF to live longer, healthier lives

We will improve understanding of the physical and mental health challenges people with CF face as they grow older, and how best to treat and manage them.

Our priorities for research

How our goals match the research priorities of the CF community

A list of the top 10 most important questions about CF research was chosen by the CF community in 2022, including the views of over 1,500 people with CF, their loved ones, members of CF teams and researchers. Each of these research priorities are covered by our research goals. Below, we've outlined the goal that most closely addresses each priority, although there are many overlapping themes.

Goal	Research priorities included
To develop effective treatments for all	What options are available for those not able to take current CFTR modulators (including rarer mutations, not eligible and unable to tolerate)? Can genetic therapies (such as gene editing, stem cell and mRNA technology) be used as a treatment for CF?
To improve diagnosis and treatment of CF lung infections and maintain lung health	What is the best way to diagnose lung infection when there is no sputum eg children and those on modulators? Is there a way of reducing the negative effects of antibiotics eg resistance risk and adverse symptoms in people with CF?
To treat all of the symptoms of CF throughout the body	How can we relieve gastro-intestinal (GI) symptoms, such as stomach pain, bloating and nausea? What are the effects of modulators on systems outside the lungs such as pancreatic function, liver disease, gastrointestinal system, bone density etc? Is there a way of preventing CF diabetes (CFD) in people with CF?
To enable people with CF to live longer, healthier lives	How do we manage an ageing population with CF? What are the long term effects of medications in CF (including CFTR modulators)? What are the effective ways of simplifying the treatment burden of people with CF?

We worked with the James Lind Alliance team at NIHR and researchers at the University of Nottingham to refresh a list of questions chosen before access to CFTR modulator medicines like Kaftrio were identified and before the COVID pandemic changed some aspects of CF care. Find out more about how these research priorities were identified on our website at www.cysticfibrosis.org.uk/researchpriorities

Goal: To develop effective treatments for all

We will develop new and better treatments that work for everyone with cystic fibrosis, and stop lives being limited by the condition.

CFTR modulator medicines such as Kaftrio have been life changing for many people with CF but they are not a cure, and they don't work for everyone with the condition. Approximately 1 in 10 people with CF in the UK are unable to benefit from CFTR modulators.

The most effective way of treating cystic fibrosis is to treat the underlying cause of the condition and we want to do this for everyone. This includes developing future CFTR modulators with increased effectiveness and reduced side effects, and finding treatments for those who are unable to benefit from them. These could include genetic therapies to make working copies of the CF protein or treatments that help the body to compensate in other ways, such as acting on other 'ion channel' proteins.

We will find out more about how the CF protein works and how this changes in cystic fibrosis, to speed up the development of new treatments.

Goal: To improve the diagnosis and treatment of CF lung infections and maintain lung health

We will find better ways to diagnose and treat lung infections, tackle antimicrobial resistance, and stop CF disrupting lives.

People with CF are at a high risk of developing lung infections, because of the thick sticky mucus in their lungs. CF lung infections can cause breathlessness and difficulty breathing. They can cause major disruptions to day-to-day life and may lead to permanent lung damage. We need effective medicines to treat these infections.

CF lung infections need to be detected and diagnosed as quickly and accurately as possible. This is important for new infections and for flare ups of long-term infections (exacerbations). Sputum samples are commonly used to detect lung infections. However, improved lung health from use of CFTR modulators such as Kaftrio mean that sputum is much harder to produce. We will support researchers to find different ways of diagnosing infections.

Bugs that cause CF lung infections change over time and can become resistant to antimicrobial medicines, meaning that medicines such as antibiotics don't work as well or no longer work at all. This is known as antimicrobial resistance (AMR). AMR is a problem for treating many different infections around the world. We will work to find new medicines that are so urgently needed to treat CF lung infections.

Goal: To treat all of the symptoms of CF throughout the body

We will improve understanding of the wide range of symptoms and complications of CF happening throughout the body and make progress in treating them effectively.

CF affects different parts of the body, and while some of these effects are already known about, we are starting to find out more about others as people with CF live longer.

For people with cystic fibrosis, CF diabetes is a difficult illness to manage alongside their other CF symptoms. Having CF diabetes increases the burden of treatment and there is a risk of serious long-term complications, such as those affecting eyesight and kidney function. The underlying cause of CF diabetes is unknown. We want to prevent CF diabetes and improve how it is treated and managed.

People with CF experience a range of gut symptoms, including small and large intestinal blockages (DIOS and constipation), bloating, nausea and diarhorrea. These symptoms can prevent people with CF getting the calories they need, be extremely painful, embarrassing and disrupt day-to-day life. Current treatments for gut symptoms are often ineffective because doctors do not fully understand why symptoms occur. We will find tailored and better ways to prevent or treat these symptoms.

We will discover more about how to treat and manage other symptoms of CF such as bone disease, liver disease and sinus complications.

We know that CFTR modulators such as Kaftrio can help improve the lung health of people with CF, but their effects outside of the lungs are less well understood. For example, what are the effects of modulators on CF diabetes and gut symptoms? We will find out what CFTR modulators do throughout the body.

Goal: To enable people with CF to live longer, healthier lives

We will improve understanding of the physical and mental health challenges people with CF face as they grow older, and how best to treat and manage them.

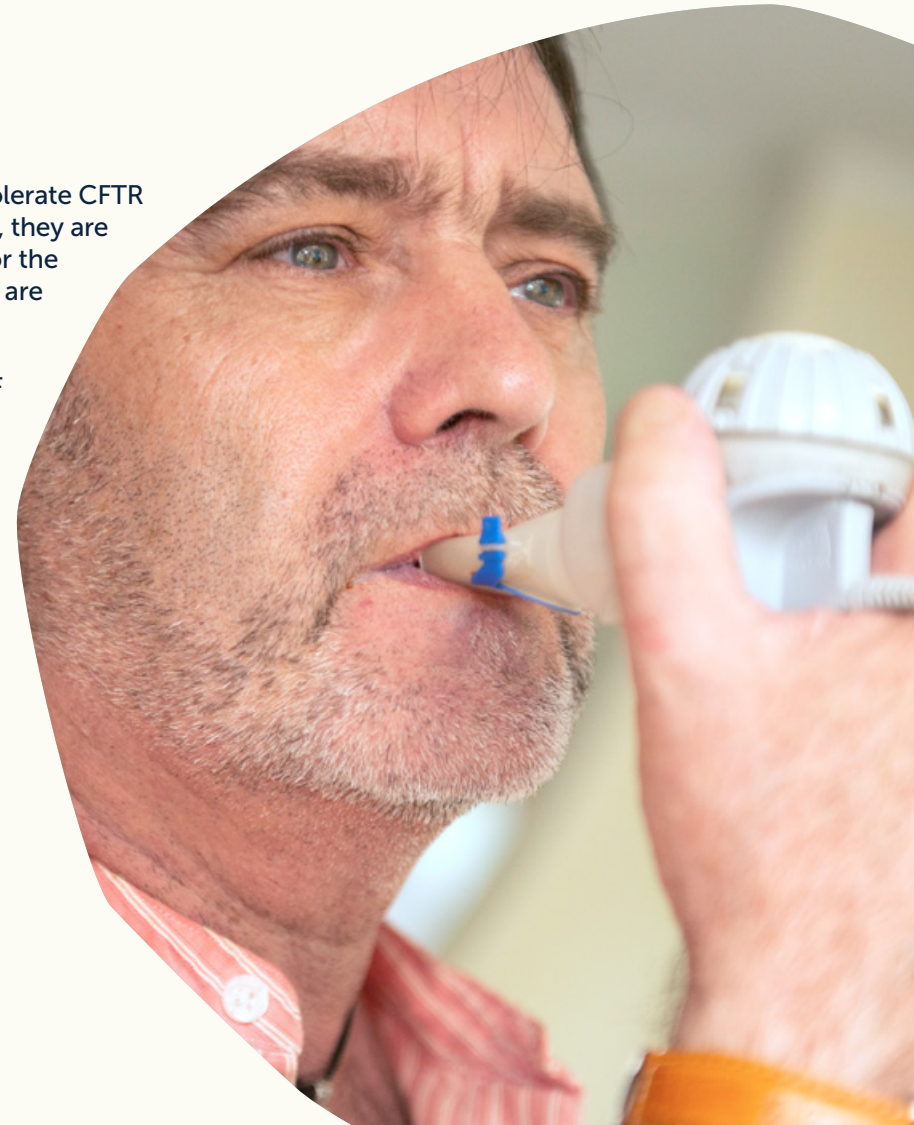
Over the last 20 years we have seen increases in the number of adults living with CF. Numbers from the UK CF Registry show that more than one in ten people with CF are now aged 40 or above (from UK CF Registry Annual Data Report 2022), as advances in treatment increase life expectancy.

We will work to unlock the many unknowns about the future health challenges that people with CF and the teams that support them may face. From ways to adjust current care and treatment, to understanding and preventing the development of diseases of an older age such as cancer, cardiovascular disease and dementia.

Everyone with CF has a daily burden of treatments to keep them well, taking up time every day and disrupting school, work and social activities. We want to reduce and simplify the treatment burden for people with CF.

For those who are eligible and can tolerate CFTR modulator medicines such as Kaftrio, they are likely to be taking these medicines for the rest of their lives. As these medicines are relatively new, the long-term effects of taking them are not understood, both in terms of improvements to CF symptoms throughout the body, and any side effects. We will improve future treatment for people with CF by finding out more about the long-term effects of modulators.

Access to modulators has increased the number of people with CF having children and we will support research to discover more about the impact of these medicines on fertility, and during pregnancy and breastfeeding.



Cystic Fibrosis Trust

Since 1964

We won't stop until CF does

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.

cysticfibrosis.org.uk

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