Cystic Fibrosis Trust

Since 1964

We won't stop until CF does

Issue 16

cysticfibrosis.org.uk

CF Life

CF care
The UK CF Registry and experiences of paediatric CF care

Fly on the wall
Ten years of exciting innovative research

Your stories
Yvonne Hughes talks stand up and her life with CF

Uniting for a life unlimited

What's inside

Issue 16 – April 2024

If you'd like to give us feedback on this issue or have ideas for what you'd like to see in the magazine, email us at **magazine@cysticfibrosis.org.uk**

On the cover: Yvonne, who features on p24
This page: Pamela, who features on p22

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Our confidential Helpline offers general advice, support and information on any aspect of cystic fibrosis, including help with financial support.

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Welcome to CF Life

In this issue of CF Life, we're really excited to share our interview with the founder of Vertex Save Us, Gayle Pledger, where we speak to her about her journey as a CF parent and how she became involved with the campaign for global access to Kaftrio.

Elsewhere in this issue, we speak to a CF psychologist to learn more about how to support young people with CF who are transitioning from paediatric to adult care. We also take a closer look at the CF community to discover the wide range of jobs and careers people with CF find themselves in.

In our ***What's on your mind?*** column, Matthew from our Helpline team answers some of your pressing questions. This month, he shares the best way to approach a friend whose baby has been diagnosed with CF.

Plus, we take a break with stand-up comedian Yvonne Hughes, who has CF, to hear about her journey into comedy and time performing at the Edinburgh Fringe Festival.

We hope you enjoy reading this issue, and please do get in touch with your ideas and feedback. We love to hear from all our incredible supporters.

The CF Life team

Uniting for a life *unlimited*

In case you missed it

60 years of progress

February 2024 marks Cystic Fibrosis Trust's 60th anniversary. Throughout the year, we will be showcasing a series of milestone events to shine a light on the huge strides that the Trust and the CF community of supporters and fundraisers have taken together over the last six decades.

From newborn screening to ground-breaking research edging us closer to the vision of a life unlimited, it's clear awareness and fundraising are making a difference.

It's a chance to recognise achievements, make people aware there's still work to be done, thank people for their support, and provide hope for a better future.

Since 1964

Research

Last year we spent £5.1 million on research. Our UK CF Conference in October was an opportunity to hear about research that we're funding and for researchers, clinicians and healthcare professionals working in the UK to network and establish new collaborations. One focus of this year's conference was addressing the recently refreshed CF research priorities.

Catch up on the highlights from the conference at **cysticfibrosis.org.uk/ukcfc**.

Support

We've created a new factsheet all about testing for potential increased risk of hearing loss with aminoglycoside antibiotics for people with CF. It explains what these antibiotics are and the risk of hearing loss, the testing process, and describes who might need the test. You can read the factsheet at **cysticfibrosis.org.uk/publications**

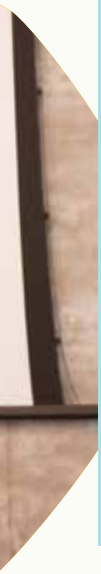
We've also updated our online content and factsheets on the sweat test and cystic fibrosis diabetes. Read more at **cysticfibrosis.org.uk/cfdiabetes** and **cysticfibrosis.org.uk/sweattest**

Young people

In September, we ran six online workshops over six weeks all about space! These workshops are part of our Building Brighter Futures programme for children with cystic fibrosis and their siblings. Space Camp offered a variety of skill-building and fun activities, from making rockets in science experiment sessions to creating pop-up space adventure storybooks in creative writing and crafts!

"Thanks for the Space Camp workshops... they went down very well and were very informative. Got their imaginations going!"

For more information about the youth programme, email **cfyouth@cysticfibrosis.org.uk**, follow us on Instagram **cftrustyouth** or visit the website **cysticfibrosis.org.uk/cf-youth**



We'd like to say thanks to the CF clinical professionals who worked with us to develop this information. You can find out all about all our information resources in our updated information resources catalogue, available at **cysticfibrosis.org.uk/information**

You can order printed copies of our information resources by contacting the Helpline.

Campaigning

Since 2022, we have published yearly reports on the experiences of those living with CF UK-wide; in summer 2023, we published our Your Life and CF report. Through your valuable insight, we have successfully campaigned for change around issues such as benefits uprating and vaping. The team conducted more research through this year's Your Life and CF survey. Keep an eye out for the upcoming report to read stories from our community and discover the latest stats on living with CF.

October 2023 saw the launch of the Support in Crisis report, highlighting the need for new government intervention to support the CF community. You can read Rita's story and how we're campaigning for the government to provide support for all people with CF in areas such as statutory sick pay and air quality at **cysticfibrosis.org.uk/costofliving**

Paediatric CF care – Insights from the UK CF Registry and our patient experience survey

At Cystic Fibrosis Trust, we regularly collect data and insights from the CF community. We use these to help inform our work with clinicians, policymakers and other stakeholders, to make recommendations about improvements in care, and to support vital research.

One incredibly important data source is the UK CF Registry. Since 2007, the Registry has been sponsored and hosted by Cystic Fibrosis Trust and collects information about the health, treatments and demographics of people with CF in the UK. Almost everyone who has CF in the UK has agreed to have their data entered into the Registry, and we are incredibly thankful for this.

> "They know my child well and always treat us in a friendly, professional way. The team use positive language but are not shy about raising difficult topics."

The data has an extraordinary impact on CF care, as it helps clinical teams to understand their own paediatric centres better and allows them to compare themselves against the national figures. The Registry data can also be used to monitor overall health trends in the CF community across the UK, to support novel research, and to provide an evidence base for new medicines.

Another valuable source of data is our patient-reported experience survey. This runs every three years in paediatric and adult care. In 2023, we reported on the findings from our latest survey of children's CF services, which found that the vast majority of children with CF and parents felt the care they receive is of high quality.

In this article, we look at some of the most interesting paediatric insights from the 2022 UK CF Registry Annual Report and our patient experience survey.

Annual reviews

Every year, people with CF have their annual review assessments with their clinical teams. Across the whole of the UK, 4,099 children and young people, whose data is on the Registry, had an annual review in 2022 at a paediatric centre. This represents 92% of those registered at paediatric centres on the UK CF Registry*.

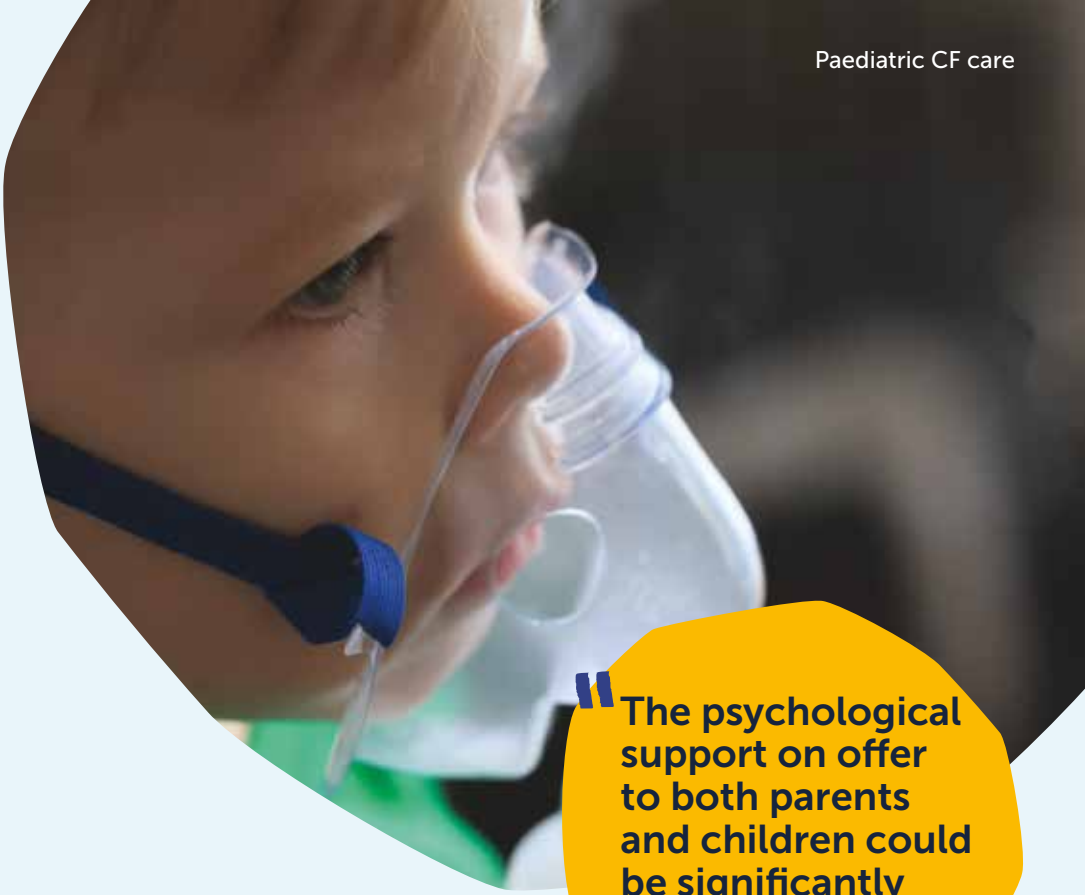
In our paediatric patient experience survey, around 9 in 10 respondents confirmed that they or their child had had an annual review, which aligns with the Registry. The survey revealed that annual reviews in paediatric care were mostly completed as outpatient appointments, with a small proportion done via video conference.

However, the survey also showed some variation in the types of assessments that are completed for annual reviews, as well as in the staff who are seen. Blood tests, chest X-rays, lung function and sputum tests were completed for most annual reviews, but the use of exercise testing and quality-of-life assessments was less consistent.

Similarly, most children and young people with CF reported seeing a doctor, nurse, physiotherapist and dietitian at their annual review, but far fewer said they had seen a pharmacist, psychologist or social worker. While not everyone with CF will need to see all members of the CF multidisciplinary team (MDT) at their annual review, it is important that there is consistent access to all specialties, including psychological and social support, when needed. Our survey revealed that this was not the case for all children and young people with CF, with some saying they had struggled to access some types of support through the CF MDT.

Cystic Fibrosis Trust is campaigning for everyone to have access to CF psychologists and social workers – not just at annual review, but as and when needed. The team are using data from the Trust's patient experience and staffing surveys to make the case to the UK government and devolved nations.

*Actively registered patients are people with CF who have consented to their data being on the CF registry and have had at least one annual review in the past three years.



> "The psychological support on offer to both parents and children could be significantly strengthened."

Improved health outcomes

In this year's annual UK CF Registry report, a noticeable change was seen in the lung function (FEV_1% predicted) of children and young people with CF. FEV_1% predicted is a widely used measure of lung health, and a healthy range is normally considered to be between 80-120% predicted.

In those aged under 18, the average FEV_1% predicted at annual review increased from 93.4% in 2021 to 95.5% in 2022. This means half the children and young people with an annual review (2,735) had an FEV_1% that was 95.5% or above in 2022. Average FEV_1% predicted increased from 93.6% to 95.8% in females and 93.3% to 95.4% in males. This is good news, as it means lung function is improving for many children and young people with CF.

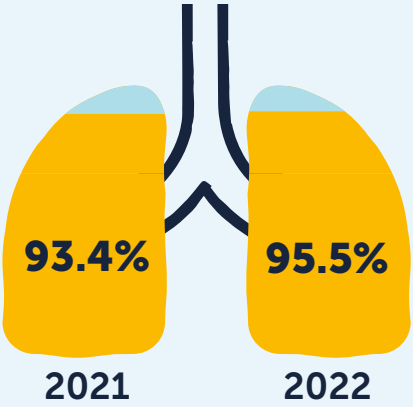
Another positive change was seen in the proportion of patients with at least one IV day, either in hospital or at home. This was lower in 2022 compared to 2021, reducing from 19.6% to 18.5% for those aged under 18 years. This reduction was seen in both hospital and home IV use, showing us that patients needed less IV treatment overall in 2022. One of the strengths of the Registry's data is that we are able to see if changes are part of a longer-term trend. Looking back further to 2017, we can see that the proportion of paediatric patients needing IV treatment has reduced by 48% in the last five years, from 35.3% to 18.5%.

4,099 paediatric annual reviews in the UK in 2022

This represents 92% of those registered at paediatric centres on the UK CF Registry

Mean FEV_1% predicted in 2021 and 2022 at annual review

Under 18 years



Intravenous antibiotics (IV) in 2021 and 2022

19.6%

of under 18s had at least one course of IV antibiotics (at home or in hospital) in **2021**

18.5%

of under 18s had at least one course of IV antibiotics (at home or in hospital) in **2022**

The results of our patient experience survey echoed these findings, as the proportion of survey respondents who said they had needed IVs in the last 12 months dropped from 32.7% in 2019 to 19.0% in 2022. For those who had needed IVs, many said these had been started without delay, and those who had received home IVs shared overwhelmingly positive experiences.

Our survey also found that, when IVs are necessary, the majority of children with CF and parents prefer to have these at home rather than in hospital. 60.5% said they would like to complete IVs at home, compared to just 21.7% who preferred to remain in hospital for IVs. This is a clear indication that home IVs should be offered where feasible, as many families prefer these.

> "We had our first course of home IVs, and it was easier and less stressful than expected. The team taught me how to administer the drugs and timings, so I felt confident to do it at home when we were discharged."

Burden of treatment

One of our research priorities was to reduce the burden of treatment for people with CF. Given the improved health outcomes seen in many who are able to benefit from modulator therapies, there is hope that treatment burden could be reduced in the future.

In 2022, 43.8% of those aged under 18 used inhaled bronchodilators, compared to 44.1% in 2021 and 45.3% in 2020. Those who faced the burden of taking combination corticosteroids and bronchodilators also showed a year-on-year reduction in the past two years, from 14.5% in 2020 to 11.3% in 2022.

While our patient experience survey revealed that the use of airway clearance equipment is still common, with 83.7% of survey respondents saying they used at least one type of device at home, studies like CF STORM are now exploring if mucoactive nebulisers might be stopped following the introduction of Kaftrio.

With the expansion of access to Kaftrio to 2–5-year-olds, it will be important to monitor what long-term impact the use of modulators could have on overall health, the use of other treatments, and the burden of care. Our ongoing data collections, including the Registry and patient experience surveys, will help us to do this, and we are thankful to everyone in the CF community who contributes.

For more information on the UK CF Registry, please visit: **cysticfibrosis.org.uk/registry**

For more information about PREMs and quality improvement, please visit: **cysticfibrosis.org.uk/qi**

To watch the recording of the CF Live event on insights into Paediatric CF care please visit: **cysticfibrosis.org.uk/cf-live**

Ten years of innovative research partnerships

Ten years ago, we created a flexible and innovative new way to support and encourage researchers to apply their knowledge and expertise to improve the lives of people with CF and their loved ones. Known as the Venture and Innovation Awards (VIAs), these grants are all about working in partnership!

"Agreeing to fund a VIA research grant can tip the balance towards an exciting CF study going ahead or not," explained Dr Paula Sommer, Head of Research at the Trust. **"It's a fantastic way of forming partnerships and supporting researchers at various stages of their careers."**

This is how two of our VIA awards have progressed CF research...

Boosting bigger studies

In 2016, we awarded a grant of £50,000 to support the CF START study. The main funding for this clinical trial was through a £1.6 million grant from the National Institute of Health and Care Research (NIHR), the government agency for funding healthcare research.

"The CF START study will determine the safest and most effective antibiotic strategy to treat babies with CF. The funding from the Trust was critical in our successful application to the NIHR to run this national study," said Professor Kevin Southern, the study's chief investigator. **"The VIA funding enabled us to undertake key preparatory meetings and community engagement. Without these funds, we would not have been able to prepare the application and early study set-up."**

Supporting more early career researchers

Studying for a PhD is one of the first career steps for researchers, and many continue to work in the same areas of research afterwards. We've been able to increase the number of people starting out in CF research by partnering with other funders to support them.

"I remember learning about the CF protein in university lectures, and I've been interested in CF research ever since," said early-career researcher Dr Ryan Marsh. **"My PhD studies were co-funded by Manchester Metropolitan University and the Trust through a VIA grant to my supervisor. I've continued in CF research, and I'm now looking at the links between the bugs in our guts and GI symptoms people with CF develop."**

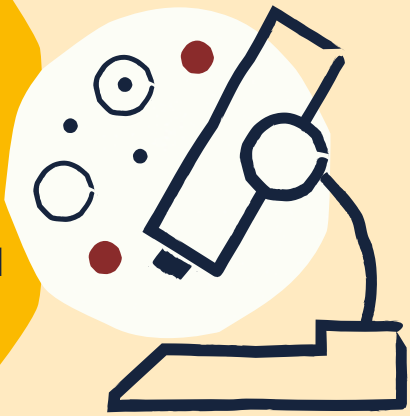
We've awarded nearly 100 grants so far, at a cost to us of £4.9m, but leveraging a huge £27.8m from other partners. In other words, for every £1 we have spent on our VIA awards, thanks to funding from our supporters, it has been matched by £5 from other funders.

If you would like to find out more about our research studies and programmes, please visit **cysticfibrosis.org.uk/researchinvestment**

Dr Paula Sommer

"Agreeing to fund a VIA research grant can tip the balance towards an exciting CF study going ahead or not. It's a fantastic way of forming partnerships and supporting researchers at various stages of their careers."

Dr Paula Sommer



Work Forwards

Forward thinking

Work Forwards is the Trust's dedicated employment programme, providing specialist information and support on all aspects of work and employment to anyone affected by cystic fibrosis in the UK. Here, we reflect on a year of Work Forwards and look to the future.

Back in September 2022, we were delighted to secure funding from Scope and the National Lottery Community Fund which meant we could launch the Work Forwards employment programme.

Through Work Forwards, we provide one-to-one support sessions. So far, we've provided over 50 sessions, supporting people with CF with every step of their career journey, from navigating the recruitment process to negotiating reasonable adjustments and flexible working, as well as challenging workplace discrimination.

People affected by CF have come to us for a wide range of reasons. Some are looking to the future, wanting support to plan careers to bring them long-term financial security. Others are looking to rebalance work and their health, making sure they have the time they need to work and look after their CF. The sessions are completely personalised and can be a one-off or a series of sessions.

Julian

"In the years before Kaftrio became available, it was sometimes challenging to fit my daily treatment regime alongside my 9 to 5 job. With CF, it's about balancing work with everything else."

Julian

One of the clear themes from these one-to-one sessions has sadly been that there are people with CF experiencing discrimination in the workplace. We're now working on some new resources to help explain CF to employers, as well as developing information to help young people with CF understand their employment rights as they take their first steps into the workplace.

In September 2023, we ran a week of activities about employment, including sessions on transferrable skills, setting up your own business, and balancing work with your health.

> "I contacted the Work Forwards team to see how they could help and what advice they could offer. With the help of the team, I learned how to deal with my anxiety and not let my nerves control me."
>
> Jonathon

Another key part of the project is sharing stories of people with CF in a wide range of jobs and careers. Research has shown that young people with CF feel their choices of work and career would be restricted by their CF. By sharing lots of different stories, we want to show everyone in the CF community that their CF won't need to define the career they choose.

Jonathan, who received support through the Work Forwards programme, said: **"I've been out of work for a long time, which really affected my mental health. I always wanted to get back into work but I just didn't see how it was possible. With countless hospital stays and hours of daily treatment, I couldn't imagine why anyone would give me the opportunity. After seeing a psychologist, I realised work was what I needed to really feel connected to the world again. I began the daunting task of applying for jobs, and soon, I started getting interviews, which both excited me and increased my anxiety.**

"I contacted the Work Forwards team to see how they could help and what advice they could offer. With the help of the team, I learned how to deal with my anxiety and not let my nerves control me – and after a few months, I was offered a job! I know this wouldn't have been possible without the help of Work Forwards. They have been imperative in offering advice and helping me prepare for interviews. Because of them, I feel more confident, and I am incredibly excited to start the next chapter of my life."

We're always looking for more stories and would love to hear from anyone in the CF community who would like to share their story about work.

Work Forwards

If you'd like support with any aspect of work or employment, you can visit **cysticfibrosis.org.uk/workforwards** or contact the Work Forwards team at **workforwards@cysticfibrosis.org.uk** or contact our Helpline on 0300 373 1000 (Mon–Fri, 10am–4pm) who will be able to refer you.

Knowing me, knowing you

Dr Anna Elderton is a Senior Clinical Psychologist at the Oxford Children's Hospital CF Centre. We caught up with her to learn more about her career, her work with CF and her top tips for a successful transition to adult care!

Dr Anna Elderton

Please could you tell us about your role?

I've worked in paediatric CF care at Oxford Children's Hospital for about ten years as a clinical psychologist. I support children with a whole range of things to help them understand and cope with their condition, such as adherence, changes in their condition, experiences of anxiety, mood difficulties, managing medical procedures, eating, and sleep. I also support parents' well-being.

What do young people and their parents often find hard about the transition to adult care?

Every young person and family are different, but we do notice themes around transition. For young people, the idea of not seeing a team of people they know well and instead having to talk to unfamiliar people about their health and potentially sensitive topics can be more daunting. Young people are sometimes unsure about the practicalities of appointments; for instance, where will I go? What will I be expected to know or do or say?

Research tells us that parents often worry more about transition than young people! Moving from a team that you know and have trusted with your child's care, often since birth, can understandably be difficult.

What are your top tips for preparing for a successful transition?

- Firstly, be kind to yourself – transition can be a big change, and some people feel more ready for it than others.
- Remind yourself that transition is a process, not an event.
- Your voice as a young person really matters in this process! Try to be actively involved in decision-making about your care.
- If you have the chance to attend a joint transition clinic, give it a go and ask any questions you have. We know that meeting your adult team and getting answers to questions reduces anxiety for most people.
- If you are experiencing more worry and anxiety than feels manageable, talk to the psychologist in your team.

For more information about transitioning into adult CF care, visit **cysticfibrosis.org.uk/transition**, or get in touch with our information and support team at **infoteam@cysticfibrosis.org.uk**

Easy exercise

We hear from Gemma Stanford, a highly specialist physiotherapist from Royal Brompton Hospital, and Pamela Scarborough, a yoga instructor and specialist CF physiotherapist, who share some yoga positions for the CF community to try at home.

Photo credits: Pamela Scarborough

Yoga can have many benefits for people with CF. There are lots of different styles of yoga, but generally, most include breathing exercises, physical yoga poses and mindfulness. Here are a couple of our favourite yoga positions for you to try:

1. Lying spinal twist offers a stretch to the upper chest. Lie on your back on any firm surface, bend your knees up and then roll them onto the floor to one side. Slowly turn your upper body and head in the other direction and let your top arm move towards the floor, creating a twist in your back. Don't twist too far, just until you feel a stretch, and then try to relax and breathe from your lower chest while holding this position for around a minute. Repeat the pose on the other side.

2. Lateral child's pose offers a stretch down your side while focusing on relaxed breathing from your lower chest. From being on all fours, reach your arms forward until your head is close to the ground, keeping your elbows straight and your hands on the floor. Slowly move your hands to one side so you feel a stretch in your side and waist. When you feel the stretch, hold it for around one minute, making sure you are breathing using your lower chest. Slowly move back to the middle and then walk your hands to the opposite side to repeat the stretch on the other side.

Researchers are currently launching the YOGA-CF study, which aims to investigate the effects of an online yoga programme for adults with CF. The study starts in early 2024 and will offer free access to yoga classes for participants.

For more information, please contact **g.stanford@rbht.nhs.uk**.

Cystic Fibrosis Trust

Imagine what you could change for the next generation with a gift in your Will

Registered with FUNDRAISING REGULATOR

A gift in your Will could help us shape a better future for people with cystic fibrosis.

Whatever your gift, this special way of supporting our work will help bring forward the day when everyone can live without the limits of CF.

If you would like to find out more about how you can leave a gift in your Will to Cystic Fibrosis Trust and help more people live the life they want for longer, please see **cysticfibrosis.org.uk/freewills**

Uniting for a life unlimited

Grand designs

Professor Dave Spring is a Principal Investigator of CF Innovation on Lung Health at the University of Cambridge. His research is focused on developing new ways to design antibiotics that will be able to treat CF lung infections, such as those caused by *Pseudomonas aeruginosa* and the NTM group of infections, including *Mycobacterium abscessus*. Sara, who has CF, caught up with him to find out more about his research.

Professor Dave Spring

> "I hope that there will be new antibiotics that work in new ways that can treat the lung infections people with CF develop."
>
> Professor Dave Spring

Sara: How did you start out in research?

Dave: Since I was 14, I've loved chemistry and making things. In my PhD research studies, I made some really complicated chemicals – but afterwards, we wouldn't use them and would just stick them in the freezer. But I wanted to make chemicals and use them for a purpose! So, I moved to designing and making new medicines.

Sara: What motivated you to work in cystic fibrosis?

Dave: Early in my career, I became good friends with someone with CF and learned about what it was like to live with it. I was always looking for an opportunity to design new medicines to help people with CF. It took a long time to realise this, but eventually, I had the opportunity, thanks to funding from Cystic Fibrosis Trust for the Innovation Hub.

Sara: Tell us about the research you're doing now.

Dave: We're looking at several different ways to design new antibiotics to make them more effective and less toxic. For example:

Antibody drug conjugates
This is a way of giving older antibiotics that have been withdrawn from use due to their toxicity a new lease of life. We chemically join an antibiotic to an antibody. When the antibody reaches the bacteria, the chemical bond is broken and only then does the medicine become active.

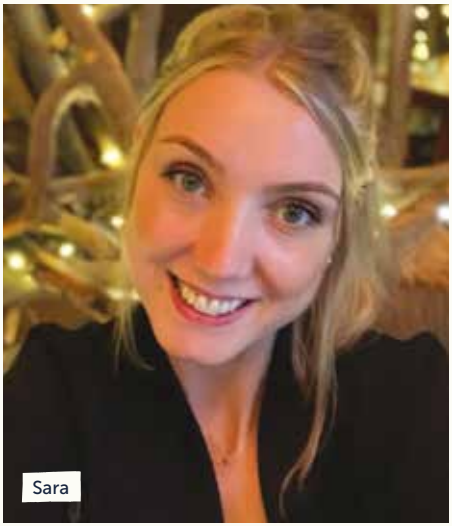
Keeping the medicines in bacteria
For a medicine to be an effective antibiotic, it needs to be able to stay within the bacteria it's designed to kill. However, bacteria have ways of preventing this! For small-molecule medicines, we're working out how to test whether any new potential medicine can get into the bacteria as early as possible in the design process.

Sara: How long does it take to develop new medicines?

Dave: It can take over ten years, depending on what type of medicine it is. Around five years of this would be working on the chemistry to make the best possible version of the medicine. The rest of the time is spent making sure the medicine is safe and effective when given to people. We've made a start on this, and we're not letting the timeframes put us off!

Sara: How will your research benefit the CF community?

Dave: It's early days, but in the future, I hope that there will be new antibiotics that work in new ways that can treat the lung infections people with CF develop.


Sara

If you would like to take part in the project to increase access to clinical trials, please get in touch with our Clinical Trials team to find out more **clinicaltrials@cysticfibrosis.org.uk**.

For more information about CF clinical trials, visit **cysticfibrosis.org.uk/clinicaltrials**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Mama Mia!

Back in 2021, a group of amazing mums, led by Pamela Millward-Browning, wanted to do something to help make a difference for their children and all those affected by cystic fibrosis (CF). With COVID-19 restrictions still in place, options were limited, but the group of 14 'CF Mamas' decided to each cover 100km over a six-week period, raising over £16,000 for the Trust in the process.

The group reunited the following year for another exercise-based challenge, and their fundraising again exceeded expectations, reaching the dizzy heights of £60,000 across the two years.

We are so proud and excited to see the return of CF Mamas in 2024, which is expanding to become CF Mamas and Papas and is expected to be even more of a bumper year to mark Cystic Fibrosis Trust's 60th anniversary.

The challenge will officially launch next month and will take place place in the four weeks leading up to this year's Wear Yellow Day. We'll be sharing more details on social media, so be sure to follow us to find out more!

Talking about setting up CF Mamas, Pamela said: **"I started the CF Mama fundraisers a few years ago after speaking to some special CF mummies. We wanted to do something as a big group and not only raise very important funding but also awareness. Cystic fibrosis is mind-boggling on the best of days just for us parents, so we wanted to make it our mission to really give people a window into our lives. To show the daily treatments and constant anxieties but also the joys in our little CF miracles.**

As parents, we'll do anything for our children, but the CF community is something else. Due to what our little ones have to go through with treatments, medications, constant infections, procedures and surgeries, we fight harder than most have to.

The CF Mamas fundraiser will be back this year, and with your support, it will be even bigger and better! We'll be opening it up to CF Papas so it can involve the whole family, a massive community who fight so fiercely for their babies."

> **"We'll be opening it up to CF Papas so it can involve the whole family, a massive community who fight so fiercely for their babies"**
>
> Pamela

A huge thanks to all the CF Mamas! We appreciate all you do to raise awareness and funds for Cystic Fibrosis Trust. If you're already taking part in an event on behalf of the Trust or want to organise your own, get in touch with us at **events@cysticfibrosis.org.uk**

Standing up to CF

Yvonne Hughes, a stand-up comedian, writer and producer from Scotland, performed at the Edinburgh Fringe Festival for the first time in 2023. Here, she talks about growing up with CF, her experience of Kaftrio and her newfound passion for comedy.

I'm Yvonne. I'm 50 years old and live in Glasgow with my partner.

I was diagnosed with cystic fibrosis at three months old. I was failing to thrive as a newborn, and I had a distended tummy, so my mum knew something wasn't right. My parents took me to hospital, and I was eventually diagnosed with CF.

Before Kaftrio, my health was in decline. I had to give up work, and I was in the process of being assessed for a transplant. I was on IV antibiotics every four to six weeks, and obviously, my mental health wasn't great. I wasn't meeting up with friends and was relying on my family to do a lot of things for me. I was also on oxygen for exercise.

I was 48 when I started Kaftrio. I'd been on Orkambi and then Symkevi on compassionate grounds. Then, as soon as it was approved in Scotland, I moved on to Kaftrio. I remember the day I started my first dose – a man came to the door to deliver my Kaftrio, and I said to him, "you're about to save my life," and he smiled. I even took a picture of him.

Whenever I was in hospital, I was always writing and turned to comedy as my escape. So, a year after starting Kaftrio, I decided to take an eight-week course, and by the end, we had to write five minutes of stand-up and perform it. I did, and I just loved it. I was laughing out loud each week with these new people I had met. I wish I had done it when I was twenty, and I might have made some money by now! But being able to perform at the Fringe this year was amazing. Edinburgh is always on the horizon when you're doing comedy, so it's wonderful that we got to go.

My comedy stems from feminism and what it's like to be a woman. I don't really talk about my CF because it's too niche, but I have created a show with two other comedians who are disabled. I could say I'm a disabled comedian, but I don't ever see myself as having a disability.

I'm living independently now, and my partner and I are also planning to get married at some point, which is lovely! I'll continue working until my stand-up starts to pay me, but my dream is to make a career out of comedy one day.

"I remember the day I started my first dose – a man came to the door to deliver my Kaftrio, and I said to him, "You're about to save my life.""

Yvonne

Do you have a creative hobby or passion you'd like to share in the magazine? If so, get in touch with us at **stories@cysticfibrosis.org.uk**

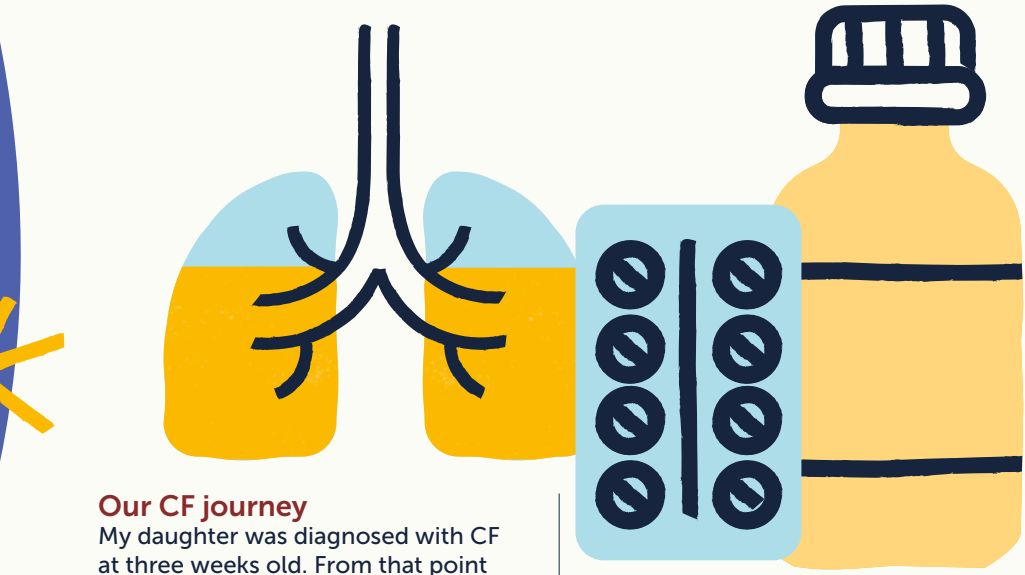
The Right to Breathe

The campaign for access to modulator treatments in the UK, has been long and challenging. At the Trust, we have been working alongside the CF community to ensure everyone who can benefit from life-saving drugs has access to them.

CF impacts lives in every part of the world. During the pandemic, the Trust worked with CF organisations on every continent to strengthen global understanding of COVID-19 on people affected by CF and to provide the best information and support. That experience highlighted that we are one CF community, but also how much the experience of living with CF can vary, particularly when it comes to access to specialist care and the latest treatments. Since the UK gained access to Kaftrio, we have asked Vertex to take urgent action to ensure that everyone who could benefit from these life-changing drugs can do so.

In this article, Gayle Pledger, co-founder of Vertex Save Us and mum to two daughters, one of whom has cystic fibrosis (CF), shares her reflections on campaigning for global access to modulators.

> **"Kaftrio has had a transformational impact on many people in the UK. It's vital that future access is secured for those who can benefit – in the UK and worldwide."**
>
> David Ramsden,
> Chief Executive of
> Cystic Fibrosis Trust



Our CF journey

My daughter was diagnosed with CF at three weeks old. From that point onwards, she was put on lots of medication and a strict medical routine to maintain her health. We literally started her on Creon® the day after her diagnosis; it was all very fast.

I very quickly became an expert in the world of CF, as you do when your child has a new diagnosis. I found out everything I could about CF and any treatments in the pipeline that could help her.

In 2015, after turning 11, my daughter's health started to deteriorate after picking up nontuberculous mycobacteria (NTM). From then on, she needed to have a two-week admission for intravenous antibiotics every three months. It was really disruptive and distressing for her.

At the same time, pharmaceutical company Vertex's drug Orkambi was approved in America. While it wasn't yet available here in the UK, we managed to get access to Orkambi on compassionate grounds just after my daughter's 12th birthday.

While it wasn't a miracle drug for her, and she still had to have regular IVs, Orkambi definitely stabilised her health and gave her a much better quality of life. It also saved her from having to have a feeding tube after she experienced severe weight loss due to the NTM.

But after months of her having access to Orkambi and seeing the rest of the community struggling without it, it seemed wrong to sit back and do nothing. So that's when I first got involved with campaigning alongside many other incredible CF families who worked hard to gain access to Orkambi. It was a great relief to us all when that happened.

Then, in July 2020, my daughter was able to start Kaftrio, which completely transformed her life. She hasn't had a single hospital admission in four years now. She is much healthier; we never hear her cough anymore, and she's no longer on oral antibiotics or nebulisers. It has changed everything. She's now 19 and studying for a journalism degree.

Deciding to keep fighting

Knowing how hard it was for us to initially get access in the UK, and then the impact Kaftrio has had not only on my daughter but so many others across the UK, has spurred me on to keep fighting and campaigning for global access to the drug.

Seeing how much harder it is for other countries, especially lower-income countries, to gain access when we struggled so much as a high-income country left me with a strong sense of moral responsibility.

Setting up Vertex Save Us

Three of us launched Vertex Save Us in October 2020. We could see that high drug pricing was a major block to access in many countries around the world, and as a result, we wanted to put pressure on Vertex rather than governments and health systems to pay these high prices.

Within a few months, we had 3,000 members from more than 60 countries on our mailing list. It was incredible. There are thousands of families out there without access to Kaftrio and other modulators, and I just wanted to use my experience to help them.

Changing focus

In February 2023, we decided to join forces with UK-based patient-led organisation Just Treatment to set up a collaborative campaign called Right to Breathe, a global coalition of CF patients, families and advocates fighting to secure worldwide access to these lifesaving CF treatments.

The main aim of Right to Breathe is to empower CF families who are directly affected by giving them the tools to push national decision-makers to override Vertex's patents on these life-changing but costly treatments if an immediate deal cannot be reached.

I could see the huge potential for this campaign as we had so much patient support and backing from families and clinicians worldwide. With all these voices, I knew that we could bring people together to make a difference.

We launched the Right to Breathe campaign with coordinated actions in four countries across four continents on the same day.

Gayle (middle) and members of Right to Breathe

Our goal was to try and push governments to override intellectual property protections and issue compulsory licences to allow low-cost generic versions of Kaftrio to be imported or made locally.

Of the four countries pushing their government to apply for compulsory licences, two of those countries – Brazil and Ukraine – now have some form of access. Brazil announced a commercial agreement with Vertex in 2023, and patients should be getting Kaftrio anytime now. So that's amazing. We are now working with patients in other countries to make similar moves for compulsory licensing.

During our latest call with Vertex representatives in October, we were deeply concerned to hear of the lack of progress with agreements for access in lower middle and low-income countries. So, we've again asked them to drop their patents in those countries to allow cheaper generic manufacturers to supply the drugs. However, at the moment, they won't agree to do that.

Instead, they say they are piloting a donation programme in a small number of countries and we are waiting for more information on how this will work. This also leaves some higher-income countries without access.

We are appealing to Vertex to take immediate action to ensure all patients have access as soon as possible. It's been more than four years since the US regulator, the FDA (US Food and Drug Administration), approved Kaftrio, so the fact that many countries still don't have access is not good enough.

Importance of campaigning

I believe that campaigning for global access is so important because it empowers people and gives them a voice. It also shows that the system is broken. This system gives a lot of power to pharmaceutical companies, and while some behave responsibly and find ways to get their drugs to everyone regardless of wealth or geography, not all of them do.

High drug prices can also cause problems in high-income countries like the UK and the US, where there are still issues with access to Kaftrio playing out. It's so important that we stand up and ensure that profits are not put before patients.

> **"I believe that campaigning for global access is so important because it empowers people and gives them a voice."**
>
> Gayle

Once these drugs are developed, of course, drug companies should be rewarded financially for their efforts, but they should then be made accessible to everyone. People should not be left dying without access to the drugs that they desperately need.

Looking to the future, I hope Vertex will begin to do things differently. We know that it is possible, and I'm sure that we all agree that everyone, everywhere, has the right to breathe.

For more about Right to Breathe, head to **www.righttobreathe.net**

Head to our website **cysticfibrosis.org.uk/nice** for the latest updates on access to medicines.

In response to the issues raised by Gayle, a Vertex spokesperson said:

"We have been able to make our CF medicines available to eligible patients in over 60 countries on five continents around the world...we recognise that there are people with CF who could benefit from our medicines who live in countries where access challenges are significant due to economic constraints and/or inadequate healthcare infrastructure. In 2022, we initiated a medicine donation pilot program for CF patients in lower income countries. The donations pilot program provides our triple combination therapy (ivacaftor/tezacaftor/elexacaftor) at no cost to eligible people with CF. At present, the pilot program includes 12 countries across four continents, with our efforts in these countries at different stages of evaluation and implementation. The learnings from this pilot will inform the future of this program, including the potential for expansion to other lower income countries."

What's on your mind?

Our Helpline Manager Matthew answers some of your questions about life with cystic fibrosis.

My friends have just had a baby who's been recently diagnosed with CF. I want to ensure I'm supporting them in the best way possible. Do you have any advice to help me?

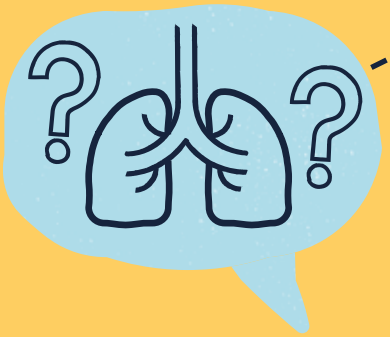
Matthew's answer: Parents have told us that the support of friends and family is really important. Often, people don't know what to say and may accidentally say insensitive or hurtful things.

Sometimes, people are so worried about what to say that they say nothing at all, which can also be hurtful. The baby is a new life to be celebrated, so while there can be difficulties, the typical responses to a new baby are appreciated.

Parents have told us that practical support is also very valued, such as help with cooking, doing the school run, taking care of siblings or picking up a prescription or shopping.

Parents often feel unable to have a break because babysitters are concerned about the care and treatment a baby with CF needs. So, being able to care for the baby, even for a short time, could be incredibly supportive to the parents.

Finally, learning about CF is also a great way of showing your support – you can find lots of information on our website. Understanding what is happening to the family will help you to support them.



If you have any further questions about CF, please feel free to contact our Helpline:

Call 0300 373 1000 or 020 3795 2184, Monday–Friday 10am–4pm

Email helpline@cysticfibrosis.org.uk

Chat with us on Facebook, Twitter or Instagram

Message us on WhatsApp on 07361 582053

What YAG means to me

Hi everyone, my name is Tilly. I'm 15, and I have CF and CF diabetes. Today, I want to talk to you about the Trust's Youth Advisory Group (YAG) and what it means to me.

One year ago, I entered my first YAG Teams call... and it made me see my conditions in a whole new way.

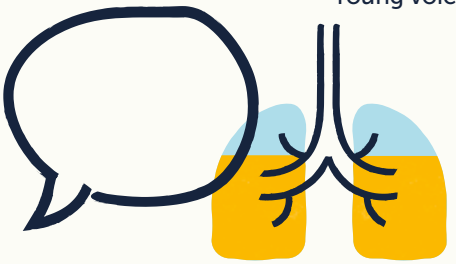
Living with CF comes with so many obstacles, but personally, I think one of the hardest things for a young child to understand is that you can't go near people exactly like you because people with CF are advised not to meet. It sounds dystopian, doesn't it? Six feet apart from the only people who will ever truly understand your health. It's really difficult to embrace your life, health and ultimately your future when you feel so completely isolated.

> **"CF is the biggest part of my life, and I've never had it in common with anyone before. It's a strange and exciting feeling to finally have that."**
>
> Tilly

In most cases, that loneliness could be fixed by meeting someone who understands how difficult it is to live with this condition. But with CF, it's not that easy. Cross-infection gets in the way and makes our lives difficult – haunting us with promises of bacteria, infection, and hospital.

But some people know exactly how I feel every day, and they live through the same experiences as me, but until now, I didn't know how to connect with them.

This is where YAG comes in and makes the loneliness disappear. Suddenly, I have a beautiful support network and community of young people exactly like me – and I can actually talk to them! Every meeting comes with feelings of relief and happiness. It's something that I'm excited to talk about and share. CF is the biggest part of my life, and I've never had it in common with anyone before. It's a strange and exciting feeling to finally have that.



Along with a mixture of feeling supported and listened to, I also feel incredibly proud because we use our meetings to help other people living with CF. The projects we do are all varied and interesting, and everyone works together to create something we're all proud of. One of our previous projects was called 'When I Grow Up', where we got adults with CF and cool jobs to talk to us about how they followed their dreams. We wanted to inspire young children with CF to do anything their heart desires, and it was a very successful project. I also took over YAG's Instagram account to promote the project, and it was really fun!

YAG has inspired me to look for the positives in my condition and not be afraid to complain about the negatives. I'd always put on a brave face in front of my family and friends because I was scared of stressing them out. However, YAG has lifted a weight from my shoulders, and I can laugh and let it all out. I can just be myself with no worries.

After a year, it's taught me so much about myself, my conditions and how I should take care of myself. It's truly an honour and a pleasure to be part of it!

We're always looking for new people to join us, so if you're 14-25 years old and would like to get involved, drop us an email at **cfyouth@cysticfibrosis.org.uk**.

Day in the life

24-year-old Jack Norris shares a typical day in his life as an actor with CF! Over to you, Jack!

1

I start each work day by waking up at 7am. From there, it's shower, morning treatment and breakfast in rapid succession!

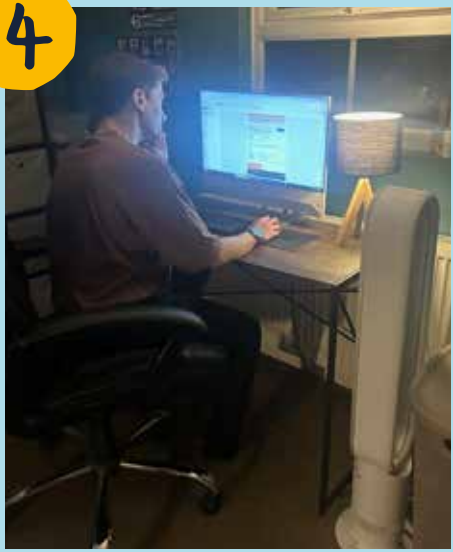
2

Due to the free-flowing nature of my career, my workplace changes regularly. The commute is a chance to read the to-do list and be truly present.

On this particular day, I was working with Caroline Quentin and David Glass on an upcoming project. The nature of my work is constantly changing.

3

4



As a freelancer, there is always more I could be doing, which has its challenges! Evenings are a good time for me to tie up loose ends and network.

5

Though I love what I do, it is often draining. Time to chill out is essential for me to re-ground and ready myself for tomorrow.

Above career, my health is everything. My day ends with doing my evening treatment and spending some time with my family. Sometimes, a home workout.

6

If you would like to share your story, please get in touch at **magazine@cysticfibrosis.org.uk**

Cystic Fibrosis Trust

Do you know about all the ways we can support you?

From providing a listening ear from someone who understands CF to need financial support through difficult times, we're here to help anyone affected by cystic fibrosis in the UK.

- Benefits advice
- Welfare grants
- Peer support
- Student support
- Help with work and employment

If you need support, contact our friendly Helpline team by phone, email, WhatsApp or on our social media channels.

Helpline@cysticfibrosis.org.uk
Phone 0300 373 1000
Whatsapp 07361 582053
(Mon–Fri, 10am–4pm)

Whatever you're dealing with, you don't have to face it alone. **We're here for you.**

Uniting for a life unlimited