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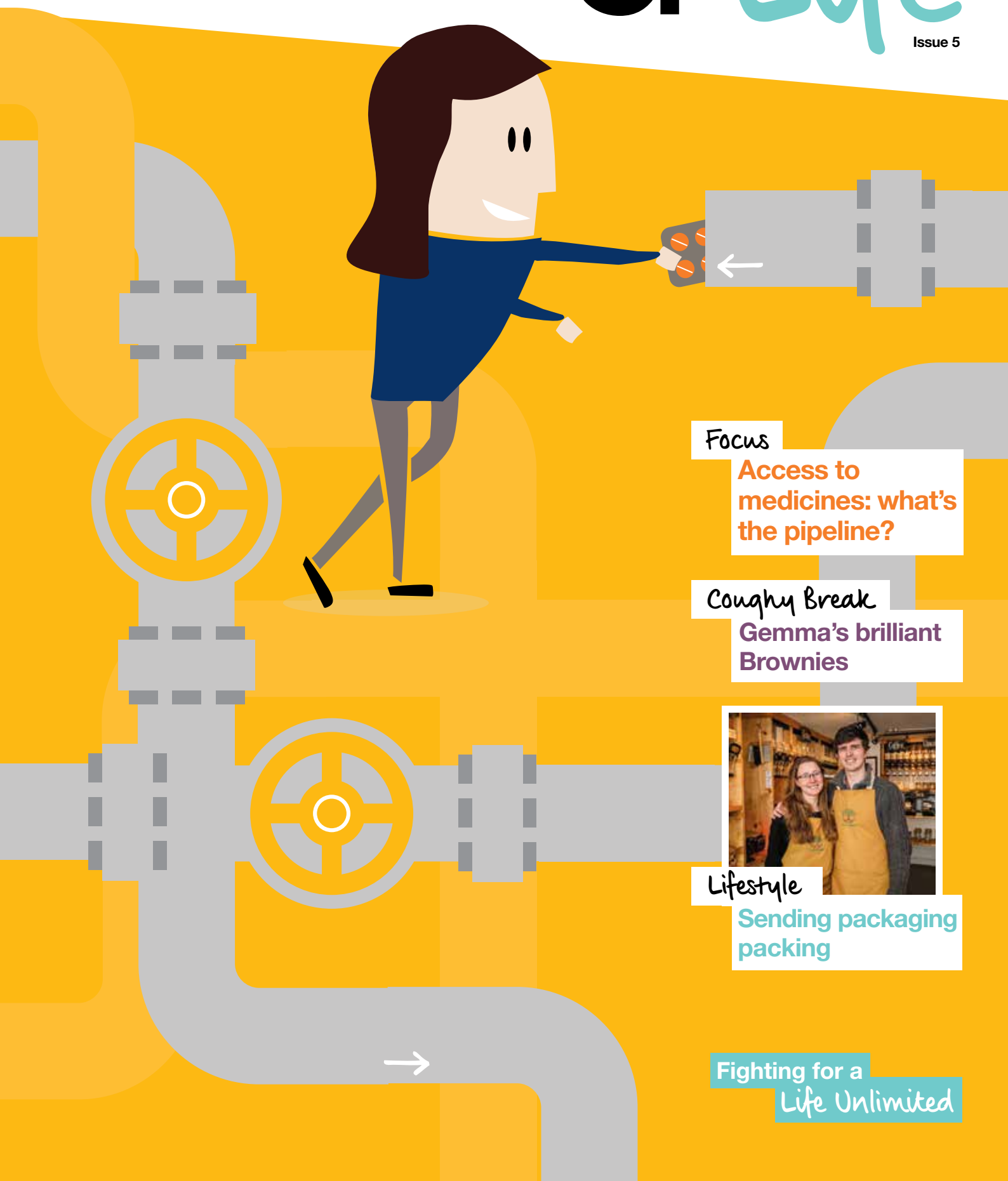
CF Life

Issue 5



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Access to medicines: what's the pipeline?

Coughy Break

Gemma's brilliant Brownies

Lifestyle

Sending packaging packing

Fighting for a Life Unlimited

Cystic Fibrosis Trust

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Useful contacts

Donations
T: 020 3795 2177
E: supportercare@cysticfibrosis.org.uk

Events and fundraising enquiries
T: 020 3795 2176
E: events@cysticfibrosis.org.uk

Cystic Fibrosis Trust helpline
T: 0300 373 1000
E: helpline@cysticfibrosis.org.uk

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

All magazine correspondence should be sent to:

CF Life Editorial Team,
Cystic Fibrosis Trust, 2nd Floor,
One Aldgate, London, EC3N 1RE

magazine@cysticfibrosis.org.uk

Welcome to CF Life

In this issue, we’re going in-depth on the fight for access to medicines. You may have taken part in a clinical trial, or perhaps signed the community petition calling for #OrkambiNow – but how does it all fit together?

The drugs pipeline runs all the way from the lab to the dispensary, and takes in research, clinical trials, appraisals – and often hard-fought campaigns – before reaching those who stand to benefit.

Keeping that pipeline flowing requires a diverse spectrum of people and skills to come together. In our main feature (p6) we present an overview of what the access pipeline looks like, and the different stages that make up the journey. On p16 we focus on a grassroots campaign and hear how members of the community and their MPs can work together to bring about change.

Our second feature (p22) looks back at some of the achievements from the last five years of research at the Trust, made possible by our fantastic supporters. We will also bring you some amazing stories from people across our community, like the brilliantly talented Jade (p26) and the inspirational Ella (p15).

You may have noticed that this issue comes in a recyclable envelope instead of a plastic bag. Thank you to one of our readers for challenging us to be more environmentally friendly – we’re sure marine biologist-turned shop keeper Robin (p20) will approve too.

If you have suggestions for us like this, or ideas for what you’d like to see in future editions, let us know, at magazine@cysticfibrosis.org.uk.

The CF Life Team

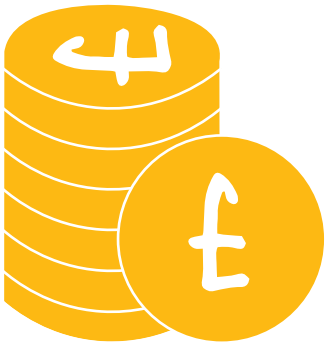
Insight Survey: What you told us

Last autumn we invited people with CF, partners and family members to participate in online focus groups and also to talk one-to-one about what we could explore in our Insight Survey, which helps us to find out what CF means to people with the condition year on year, and direct the focus of our work. The subjects raised are all either featured in this year's survey or being explored in other ways – for example, in our Information and Support Team's work.

We took some of the most striking data from the 2018 CF Insight Survey and created an 'at a glance' report. Read on for the headlines, and be sure to keep an eye out for the full Insight Survey report, which will be released this month.

1,095

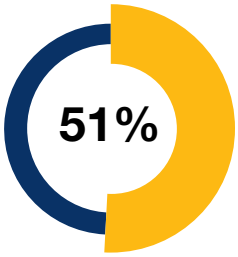
people completed 100% of the survey, while 400 more surveys were 'substantially completed'



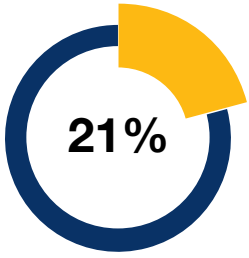
77%

of adults with CF experienced at least some concern about the financial burden of their condition.

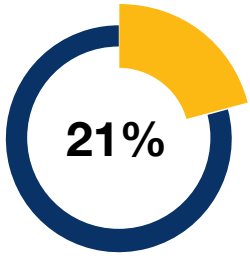
Of the survey responses:



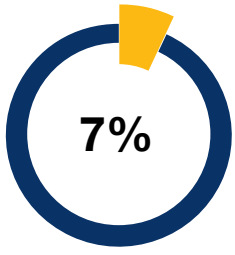
were from parents of people with CF (558)



were from relatives and friends of people with CF (230)



were from people with CF (230)



were from partners of people with CF (77)

If you could change one thing...

When people with CF were asked what one thing would improve their lives, they said:

- **10%** Prioritisation of research
- **12%** CF to be better known and understood
- **19%** Being treated with empathy and understanding
- **21%** Access to medicines
- **21%** Help with the financial burden of CF

When asked the same question, partners were the highest percentage of respondents that felt CF being better understood would improve their lives.

Parents of under 16s with CF felt most strongly out of all groups that access to medicines would improve the lives of their children.

Concerns

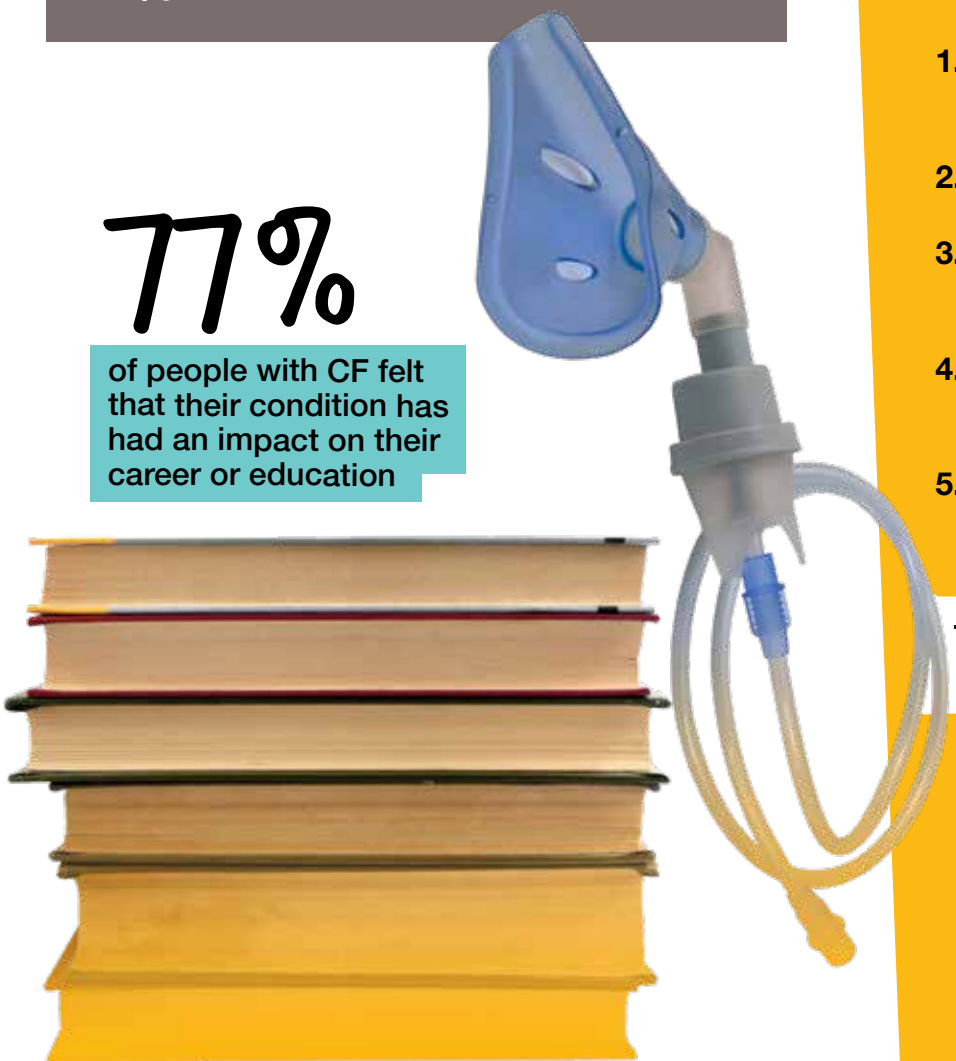
We asked people with CF what their concerns relating to care were for the next 12 months. They said:

1. Access to new treatments and medicines
2. Cross-infection
3. Getting a hospital bed when they need one
4. The additional costs of care like travel and prescriptions
5. Access to current medicines

To read the full report visit cysticfibrosis.org.uk/insightsurvey

77%

of people with CF felt that their condition has had an impact on their career or education



Access to medicines: what's the pipeline?

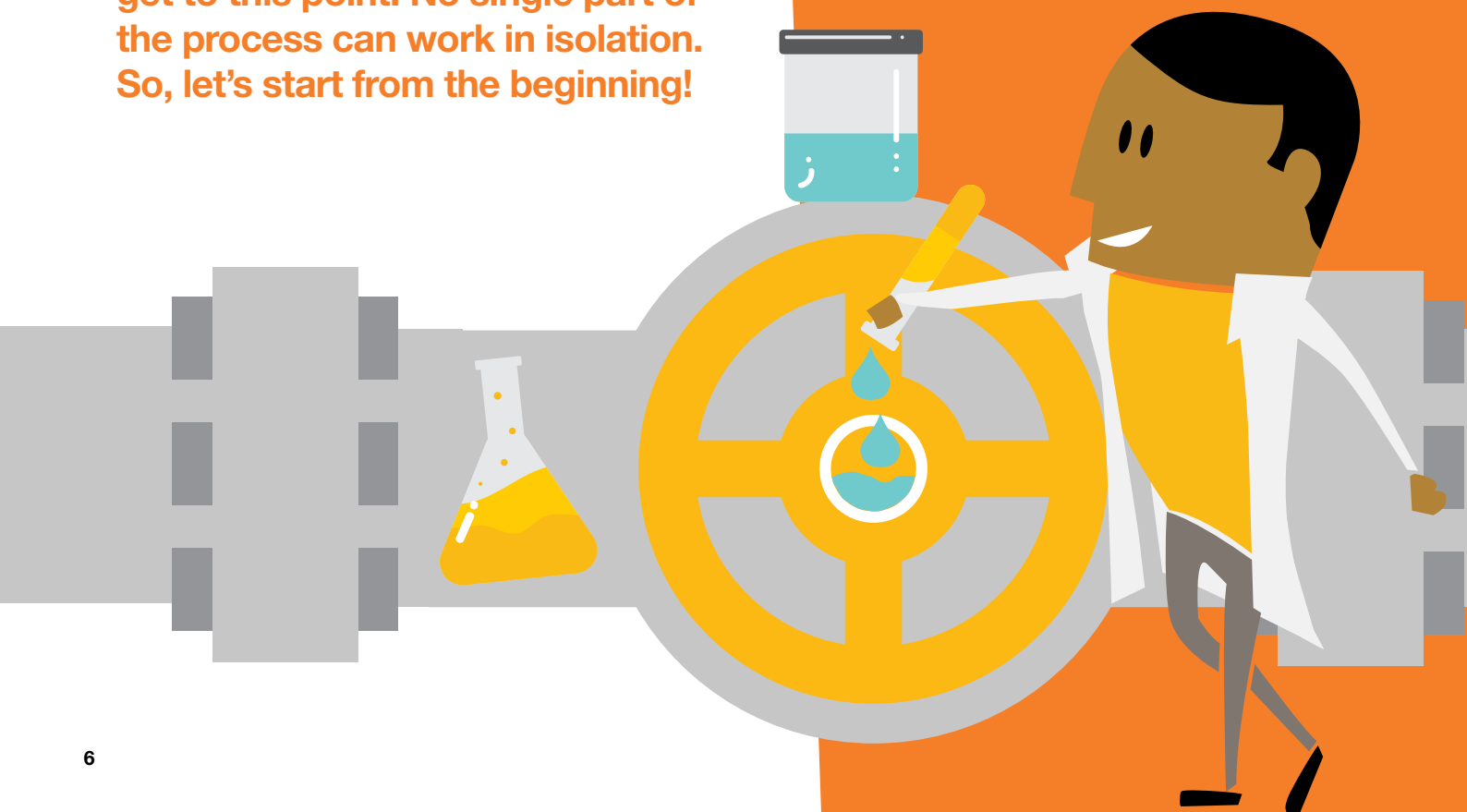
Knocking on the door of 10 Downing Street or standing shoulder to shoulder with campaigners outside Government buildings can be game-changing moments in a campaign for access to medicines for people with CF, but it takes many steps to get to this point. No single part of the process can work in isolation. So, let's start from the beginning!

In the lab

At the heart of our mission is investment in a variety of research avenues that benefit everyone living with CF, now and in the future. Framed by our new five-year research strategy (more on page 22!), one such focus is on disease-modifying precision medicines that target the root cause of CF rather than the symptoms of the condition. This kind of research has changed the outlook for CF and bridged a huge gap in the understanding of how different mutations affect the CFTR protein, and what interventions are required, moving away from a one-size-fits-all approach towards tailored treatment and care.

Several pharmaceutical companies are looking at a pipeline of therapeutic drugs and the Trust is working to build relationships and ensure that researchers and industry alike remain interested in cystic fibrosis.

This includes building partnerships with funding agencies, leveraging funding from the Life Sciences Industrial Strategy by joining forces with industry and acting as a neutral broker between academic scientists and industry. Maximising our impact with a broad range of investments in research will continue.



We look to get the most from your money by investing in a diverse research portfolio, including our Strategic Research Centres, the Innovation Hub, SmartCareCF, the UK CF Registry and our Clinical Trials Accelerator Platform, with all of these bodies of work securing and promoting access to medicines and high-quality care.

Clinical trials and you

According to a 2015 article in The Pharmaceutical Journal, for every 25,000 compounds (drugs) that begin development in the laboratory, only 25 are tested in humans and only five reach the market. With so few drugs making it to market, it's vital we support and engage with clinical trials wherever we can.

Cavan Arrowsmith took part in the clinical trial for tezacaftor/ivacaftor, the double combination therapy known as Symkevi (or Symdeko in the US).

"I know that participating in clinical trials will benefit the present and future CF community in finding new, more efficient medications."

Understanding mutations

Class I
Deletions in genetic coding and a premature end to the protein chain stop CFTR production. Eg W1282X.

Class II
Misfolding of the CFTR protein causes it to be defective and destroyed before reaching the cell surface where it would act as a channel to keep a flow of salt and water. Those that do reach the surface do so in a reduced form. Eg F508del.

Class III
Incorrect building blocks in the protein chain result in a gating defect, meaning that opening of CFTR channels is significantly reduced. Eg G551D.

Class IV
Changes in the structure of CFTR proteins affect the ability to move substances through pores in the channel. Eg R117H.

Class V
Reduced amounts of functioning CFTR protein are made due to disruption in the process. Eg A455E.

Class VI
Mutation causes a high turnover of CFTR proteins, so the ones at the cell surface are unstable and can be lost or removed. Eg N287Y.

Five things you should know about clinical trials

1. You do not need to wait to be invited to participate – you can be proactive and seek trials information yourself, either from the Trials Tracker or by talking to your CF team. (visit cysticfibrosis.org.uk/trialstracker)
2. Trials run across the UK (Scotland, Wales & Northern Ireland included) – even if a trial is not running at the CF centre where you receive your standard care, you can be referred.
3. There are trials available that are not dependent upon mutation – some for new medications, some for new devices or techniques (ie nebulisers or physio). Trials for CFTR modulators require few participants and are highly sought between countries and patients, making recruitment competitive.
4. Some trials can be short term (eg 4-6 weeks), and others can be completed from home or combined with routine CF care appointments, to reduce the burden of trial participation.
5. You can change your mind about taking part at any time – you do not have to complete a trial you start if it becomes difficult or time consuming.

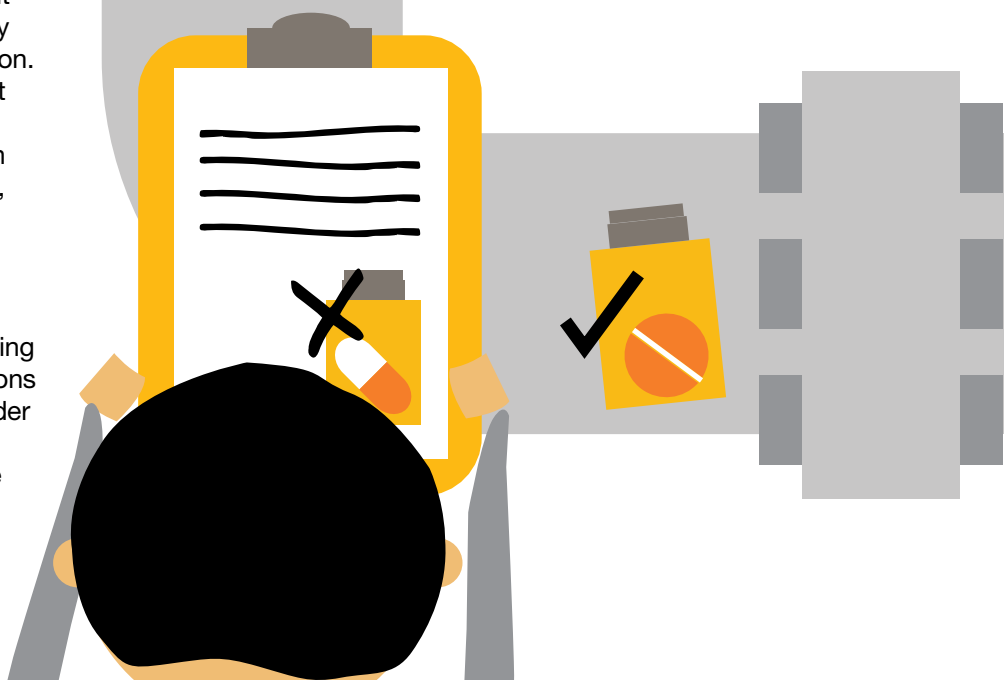
"I signed up to take part in a clinical trial to see whether any new treatments are out there that may benefit me more than my current arsenal of prescription medication. The second reason was that I know that participating in clinical trials will benefit the present and future CF community in finding new, more efficient medications, like Symkevi.

"The Symkevi/Symdeko trial came at a point in my life where I was starting to struggle with my health and I was entering a six-monthly cycle of hospital admissions for IV antibiotics. I had started to consider reducing from full-time employment to a four-day week, just so I could find the energy to keep up the fight. Symkevi changed all that within a matter of weeks. My lung function (FEV_1) had previously dropped to 79% and within four weeks of being on Symkevi it was up to over 100%.

"Clinical trials are easier to participate in than you think but there is a commitment needed to help successfully complete the trial.

"I think of clinical trial visits as receiving a full body MOT and service. I get to see the same 'mechanics' at each clinical visit, building stronger relationships with the medical staff, and as I'm monitored more frequently any result that looks awry is addressed early.

"On the Symkevi/Symdeko clinical trial I tend to opt for an early start so that the visits do not impact on my day too much. I arrive in clinic for 8am and I usually leave by 10.30am with another two months' worth of Symkevi. I have a blood and urine test, ECG, answer mental and physical health questionnaires, do a lung function test and I'm done. I also get breakfast and paid £75 for each clinic visit. It's not a bad experience at all given all the benefits and I'm getting to try a brand new, cutting-edge medicine that's helped to increase my lung function by 24%. What's not to like?!"

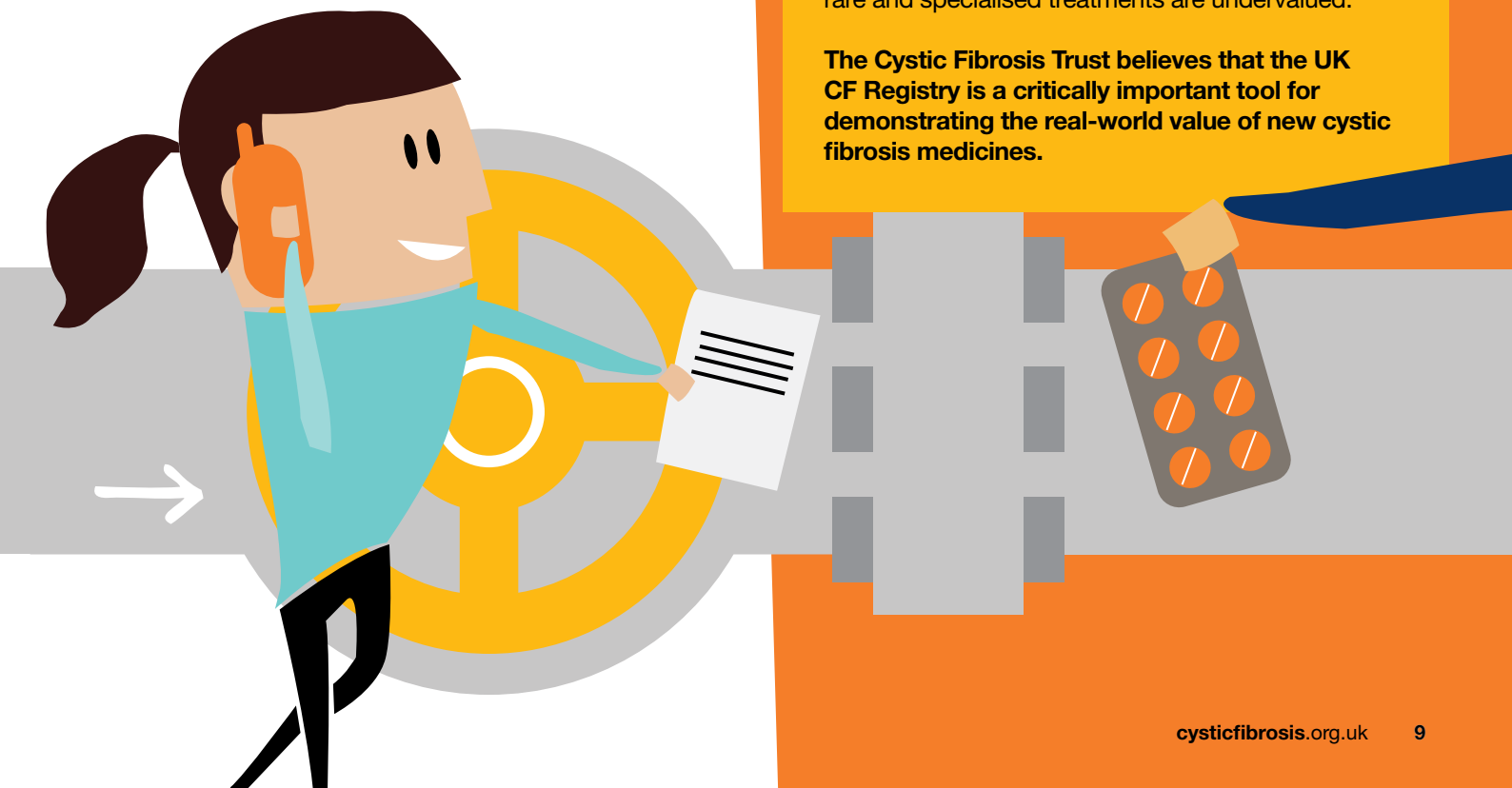


And now it's time to give your voice

The community voice is vital when it comes to sharing the experiences of living with CF with others, including key decision makers. At the time of writing, over 780 people had completed a survey to feed into the National Institute for Health and Care Excellence (NICE) appraisal of Symkevi. Once a medicine or treatment has been licensed, it must be appraised by the regulator - NICE or the Scottish Medicines Consortium (SMC) in Scotland, with Wales (All Wales Medicines Strategy Group) and Northern Ireland often following NICE. This process involves weighing up the cost of the medicine with the clinical trial data, and there is an opportunity to formally submit evidence reflecting patient experiences.

"...for every 25,000 compounds (drugs) that begin development in the laboratory, only five reach the market."

There's a long way from the lab to the shelf for most medicines, but what is important to remember is how far we have come once we have reached the point of campaigning for access to a medicine.



What is the NICE appraisal process?

1. The Government asks NICE to recommend how to fairly distribute the health and social care budget by investigating available evidence. This means NICE recommends whether the NHS in England should fund new treatments. The NHS has a legal obligation to fund treatments recommended by NICE. Each of the four nations has its own appraisal process; this one is used in England.

2. NICE reviews the clinical evidence about how well the treatment works and predicts the benefit this could have over a person's entire life. NICE also looks at the economic evidence – how much the treatment would cost over a person's entire life.

3. NICE then compares the cost and benefit of the new treatment to current treatments and a committee of experts, including clinicians, scientists, patients and economists, makes a final decision about whether the new treatment offers value for money.

NICE's methods are internationally respected but have been heavily criticised by many who argue that rare and specialised treatments are undervalued.

The Cystic Fibrosis Trust believes that the UK CF Registry is a critically important tool for demonstrating the real-world value of new cystic fibrosis medicines.

Our research partners

Venture and Innovation Awards (VIAs) help to take advantage of research opportunities and leverage external funds through partnerships with different organisations. For every £1 we've spent funding VIAs, almost £4 has been contributed by external partners!

These partners include industry bodies, research councils, the National Institute for Health Research (NIHR) and biomedical research charities. But what are we doing with these partners to capitalise on cutting-edge CF research?

Academia

Computer analysis of lung CT scans to measure lung health

Our contribution: £50,000
Partner (and contribution): Centre for Medical Imaging Computing, UCL, London (£50,000)

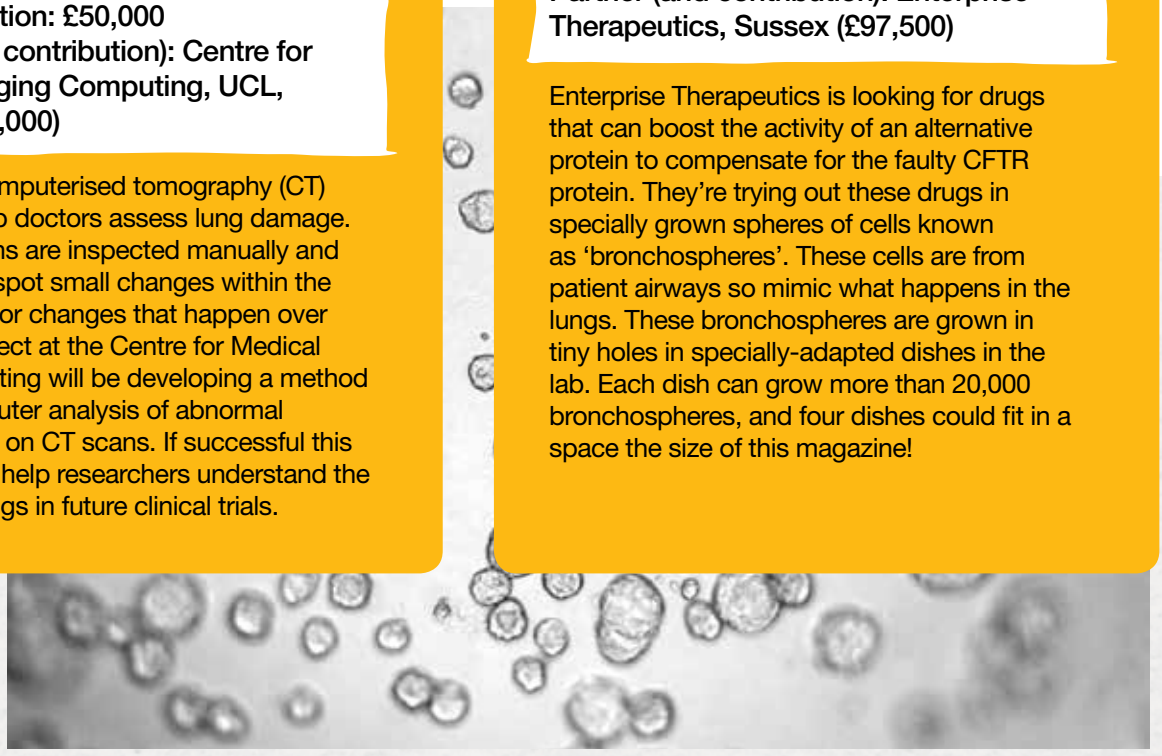
Performing computerised tomography (CT) scans can help doctors assess lung damage. Currently, scans are inspected manually and it's difficult to spot small changes within the lungs or monitor changes that happen over time. This project at the Centre for Medical Image Computing will be developing a method to allow computer analysis of abnormal airway dilation on CT scans. If successful this method could help researchers understand the benefits of drugs in future clinical trials.

Pharma

Drug screening for easier airway clearance

Our contribution: £97,500
Partner (and contribution): Enterprise Therapeutics, Sussex (£97,500)

Enterprise Therapeutics is looking for drugs that can boost the activity of an alternative protein to compensate for the faulty CFTR protein. They're trying out these drugs in specially grown spheres of cells known as 'bronchospheres'. These cells are from patient airways so mimic what happens in the lungs. These bronchospheres are grown in tiny holes in specially-adapted dishes in the lab. Each dish can grow more than 20,000 bronchospheres, and four dishes could fit in a space the size of this magazine!



Researchers from Enterprise Therapeutics

Charity

Understanding and detecting early stages of Pseudomonas infection

Our contribution: £62,000
Partner (and contribution): Action Medical Research (£124,000)

Pseudomonas initially infects the nose and upper airways before moving down to infect the lungs. Once there, Pseudomonas adapts to its living environment and becomes much more difficult to treat. This project is working to find out when Pseudomonas begins to adapt, and how to spot this taking place. The results could help doctors target infections much earlier, before they have a chance to adapt.

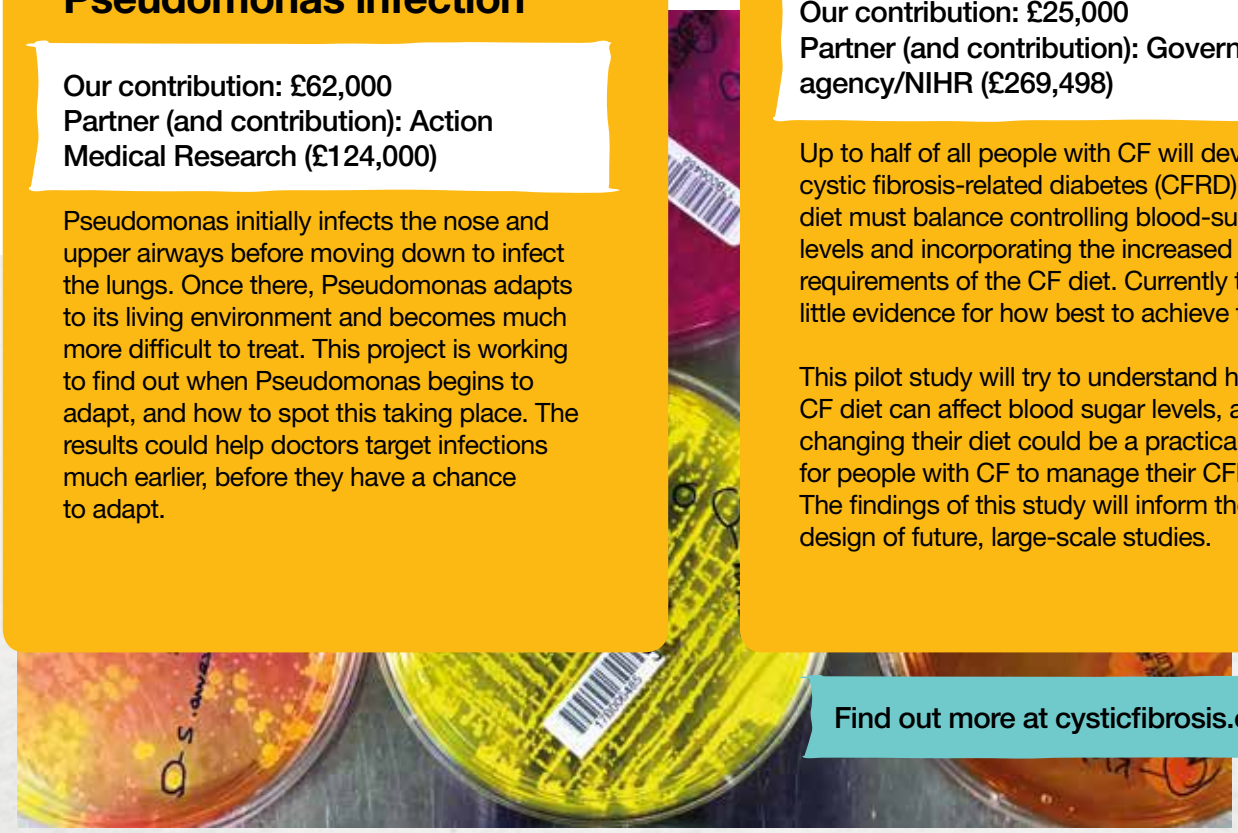
Public body

Dietary manipulation to improve glycaemic control

Our contribution: £25,000
Partner (and contribution): Government agency/NIHR (£269,498)

Up to half of all people with CF will develop cystic fibrosis-related diabetes (CFRD). Their diet must balance controlling blood-sugar levels and incorporating the increased energy requirements of the CF diet. Currently there is little evidence for how best to achieve this.

This pilot study will try to understand how the CF diet can affect blood sugar levels, and if changing their diet could be a practical way for people with CF to manage their CFRD. The findings of this study will inform the design of future, large-scale studies.



Find out more at cysticfibrosis.org.uk/VIA

Interview with an innovator: Professor Ludovic Vallier

Professor Ludovic Vallier is a specialist in regenerative medicine based at the Cambridge Stem Cell Institute at the University of Cambridge. He's also one of the principal investigators within the Trust's UK Cystic Fibrosis Innovation Hub on lung health. CF Life caught up with him over the summer to ask him about his work.

What's your project about?

"The aim of our project is to use stem cells to develop new therapies for cystic fibrosis. More precisely, we would like to use human, induced pluripotent stem cells or `iPSCs' to generate lung cells in a dish, which could be transplanted to repair damaged lungs or used to develop new ways to test for new drugs."

What are stem cells?

"Stem cells are basic cells that have the potential to grow into any cells in our body. One way to obtain them is to make iPSCs in the lab using cells in the blood, which in this case are donated by people with CF at clinic appointments. This means that the iPSCs carry the CF mutation and can thus be used to perform studies in the lab."

How long do iPSCs take to grow into lung cells?

"Producing lung cells from iPSCs takes between 20–30 days. It's a long process that requires constant attention."

What do you have to feed them on, and how often do they need feeding?

"We feed the cells every day with nutrients and factors that help them to grow and to maintain their identity. They grow very fast and thus are extremely hungry. This means we have to be in the lab every single day, including weekends!"

How can you tell that your experiments are working?

"We know that the stem cells have turned into lung cells because we can see the beating cilia (hairs on the surface of the cells that help move the mucus along the lungs) under a microscope."

What are the advantages of working in the Innovation Hub?

"The Innovation Hub is a unique opportunity to bring complementary expertise together. For example, we can benefit from the clinical knowledge brought by Professor Andres Floto, access primary samples and exchange ideas on the requirement for drug development. Such organisation is essential to facilitate information, collaboration and resource sharing. In addition, the Hub gives us the opportunity to meet on a regular basis and discuss the latest developments in our respective fields, thereby increasing our creativity."

The best bit about my research is working with my team to generate new knowledge and then using it for the development of new therapies.

The worst bit about my research is the lack of time! It would be great to have 36-hour days and 10-day weeks.

To find out more about our Innovation Hub, visit cysticfibrosis.org.uk/innovationhub

News in pictures

Inspired by William

The Anchorians Football Club raised over £4,500 for the Trust at their Charity Football Match, inspired by one of their young players, William, who has cystic fibrosis. The club has been named the FA Grassroots Football Awards National Charter Standard Club of the Year for 2018.

Online Book of Remembrance

Our online Book of Remembrance launched on 30 August, giving our supporters a special place to remember those who have died of cystic fibrosis. If you'd like to find out more, contact Michael Clark on 020 3795 2132 or email inmemory@cysticfibrosis.org.uk

Delilah, what a star!

Delilah Daisy organised a Wear Yellow Day at her school, North Clifton Primary. She gave a presentation about CF, and also sold cakes and yellow flower badges and raised an amazing £143!! All this was in support of her big sister, 19-year-old Isobel who has cystic fibrosis.

Welcome Richard

In July we wished farewell to George Jenkins OBE, who has been our Chairman since 2012, and welcomed new Chairman Richard Hunt CBE, who said: "I am delighted to take on this role at such a critical and exciting time in the world of cystic fibrosis." Welcome to the team Richard!

Amazon Smile – Give a little, get a lot

Change lives while you change your wardrobe! Visit smile.amazon.co.uk for the same products, prices and shopping features as Amazon.com, select the Trust as your charity and the AmazonSmile Foundation will donate 0.5% of the purchase price.

amazonsmile
You shop. Amazon gives.

New York and back again

Ben Coulthard and the Brigade Denison Barracks raised almost £1,000 rowing, cycling and running the distance of Buckingham Palace to New York in 52-and-a-half hours! All in memory of Ben's sister Danielle, who died before she was able to travel to New York.

Our Christmas shop is open!

Choose from our gifts and brand new cards, including a beautiful design from last year's card competition winner Louisa, and a multipack with designs from our talented runners-up.

Visit cysticfibrosis.org.uk and search 'Christmas shop'

"I was really excited to find out I was the winner of the Trust's Christmas card competition! I love penguins and think they look so cute. My lovely cousin Morven has cystic fibrosis and I hope the cards help raise lots of money to help keep her and other people with CF healthy."

- Louisa

Meet... Martin Wildman

Dr Martin Wildman

Millions are spent developing treatments to help people with CF keep their lungs healthy – but little until now on helping people take those treatments. Dr Martin Wildman, Clinical Lead at the Sheffield adult CF centre, tells us how CFHealthHub, a digital health project part-funded by the Trust, could change that.

Can you briefly explain what CFHealthHub is?

"Since 2014 around £6 million has been invested to support people with CF to work with researchers to develop a digital platform that automatically captures when nebulised therapies are taken and makes that information visible on smart phones. The individuals control this information but can choose to share it with their clinical team. CFHealthHub contains resources that individuals can use with the support of the clinical team to create habits of self-care."

How could accurate adherence data improve how people with CF and their clinical teams understand their health?

"It can be hard to remember how much treatment has been taken over past weeks and months and having an automatic record available can help the clinical team work with individuals to sort out all the factors that might affect the lung function measured in clinic. For example, if it has been hard to take a twice-daily inhaled therapy over the past two months, switching to a three-times-a-day treatment might not be the best choice. Optimum care planning should take into account lung function, weight and medication use and CFHealthHub makes this information easily available, in powerful, simple graphics at every consultation."

Where do you see CFHealthHub fitting into the wider world of digital health?

"CFHealthHub has been in continuous use since 2015 and is being used by almost 1,000 people in 22 adult centres in the UK; as such it has the potential to provide infrastructure that might support other digital health developments in the future. We are already starting to explore ways in which CFHealthHub might enhance the UK Cystic Fibrosis Registry."

"Optimum care planning should take into account lung function, weight and medication use."

– Martin

The Trust is funding other programmes looking at many aspects of digital health, like SmartCareCF. Find out more at cysticfibrosis.org.uk/smartcarecf.

CFHealthHub

Ella's story

Sporty 13-year-old (and Shawn Mendes obsessive) Ella tells us about staying motivated to keep on top of her treatments and out of hospital.

Since I was diagnosed at 11 months I have had to have operations, do hours of physio every day and take mountains of pills. At first, I was in hospital about every six weeks. At the time I didn't really understand, but it must have been horrible for my parents. One of my real struggles was finding a motivation to do physio but it turned out I knew it all along: no physio = hospital. I really, really didn't want to go into hospital!

As I've grown up and become more aware, I've increased the gap between my hospital stays to half a year, then a year, then THREE YEARS! As much as it was horrible to have to go into hospital after three years, I felt really proud that I'd managed to stay well for that long.

When it comes to needles I had a massive phobia when I was young, so I had a portacath until about two years ago, when it stopped flushing properly. But because I had got so much healthier (and overcome my phobia) and hadn't been in hospital for ages, they decided to remove it!

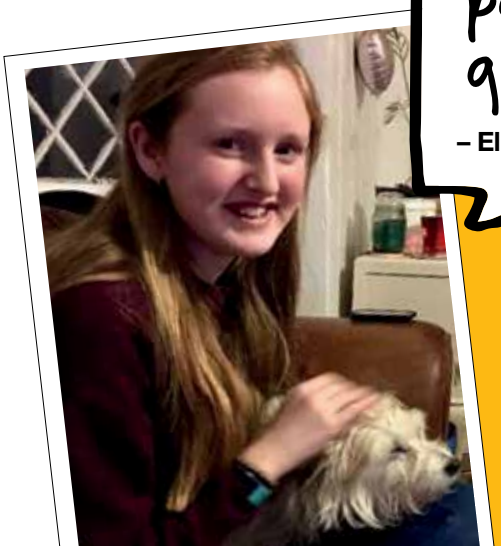
I believe I have got healthier because I'm making my physio a habit, doing lots of exercise and drinking lots of water. Exercise is a massive part of my life because I notice when I'm really fit, my chest is amazing, but when I'm really not, my chest gets worse. All my medicines work together too - if you take one part out, it all goes wrong.

Recently I have been seeing a chiropractor. This was originally because I have a 'clicky' jaw from where I ate pork crackling and tore a ligament - whoops! But then she found out about my CF and decided to try some things for my chest which have really helped open me up.

Ella is motivated to stay healthy and out of hospital

"All my medicines work together - if you take one part out, it all goes wrong."

– Ella



Putting the MP in campaigning

In the fight for access to medicines, collaboration between the community, Parliamentarians and the Trust is vital. After finding out that her daughter Annabelle had CF, Liz Brennan discovered the power of the online community in fundraising and campaigning. Along the way Liz has worked closely with her MP, Luke Hall, to raise awareness of CF and access to medicines.

Liz: "The diagnosis hit me incredibly hard. I'm a person who likes to feel in control and I felt completely hopeless in how I could make sure my daughter's future was more certain. Being online a lot, I started to see and talk to lots of families who were going through the same thing. In April 2016 we held a community fundraiser and invited our MP, Luke Hall. At that point we just wanted to engage with him, gain his support on the struggles people with CF face and explain what CF is."

Luke: "It was absolutely inspiring to hear about the challenges people living with CF and their families have to face every day, and I was very keen to support my constituents in any way I could, from attending great local awareness and fundraising events to events in Parliament.

"I have also been working with my constituents in raising the issue with the Health Minister, and was very pleased to have the chance recently to speak in a very important debate in Parliament on Orkambi. I continue to support my constituents and to monitor the progress of the crucial ongoing negotiations between NHS England and Vertex."

Liz: "The first time I met Luke, I was extremely nervous. We see these people in the news and on the TV and we seem to feel they are in a different league, but this wasn't the case in my experience. Luke is very down to earth and easy to talk to."

Luke: "As an MP, it was amazing to see how successful this campaign has been. The petition was a fantastic effort, with more than 100,000 signatures in just a few days, and the debate was one of the most successful Westminster Hall debates I have seen, with MPs from all parties showing interest.

"The different briefings and information packs that the Trust provides MPs are extremely useful: to help raise awareness of the issue, and then to keep up to date with any developments as the campaign progresses.

"I completely understand people may be hesitant to share their deeply personal experiences of living with CF with their MPs, but this can actually be really important. It gives your Parliamentary representatives the chance to see the effect this debilitating condition has on their own constituents, and can really help when pressing them to raise the issue on your behalf.

"So, if you are contacting your MP for the first time, absolutely send them an email or drop by their surgeries if they are holding them near your area, and start that dialogue."

Campaigning in numbers

40 MPs at Ian Austin's roundtable

59 MPs attended the Westminster Hall debate

5,715 signatures on Orkambi petition sent to the Welsh Assembly

117,955 signatures on petition for access to Orkambi in the UK

1,000 letters delivered to the Prime Minister on 16 May

c.200 campaigners at the 2018 protests in London and Belfast

218 UK-wide Parliamentarians engaging with the campaign for access to medicines in 2017/18

Liz's top tips for working with your MP

1. Try to put party politics to one side.
MPs are voted in by your community – a big part of their role is to try and help their constituents and represent them in Parliament. Don't worry if your MP is not a member of your political party of choice. Cystic fibrosis and access to medicines are cross-party issues.

2. Come prepared with information and be prepared to explain.
MPs are a bit like GPs – they may have a little knowledge of everything and may be specialist in one area, but are not necessarily experts on the subject of cystic fibrosis and the medicines that treat it. You have to think of it as a journey and learning curve for both you and your Parliamentarian.

3. Don't get lost in their inbox – always follow up with a phone call.
Be mindful that MPs receive hundreds of emails a day. This means we must work extra hard to get noticed – polite persistence pays off.

4. Put a face and personal story to your email.
Words in an email or letter are great for reference but can be very impersonal. Meeting your MP and looking them in the eye goes a long way towards gaining their support. Book an appointment and find out if they have drop-in surgeries. MPs want to help their local area and the people who live in it, so sometimes it's important to avoid blanket information and focus on how they could help you and your family. Show photographs and evidence to help them visualise the issues. Let's face it, CF is complicated!

5. Keep in contact.
Once you have met or spoken with your MP, keep in regular contact to update them on key movements, and meet up regularly. If you're doing any fundraising or awareness activities then invite them along. It helps to keep you in their mind.

Coughy break, shining a spotlight on the talented, creative side of the cystic fibrosis community.

Gemma's brilliant Brownies

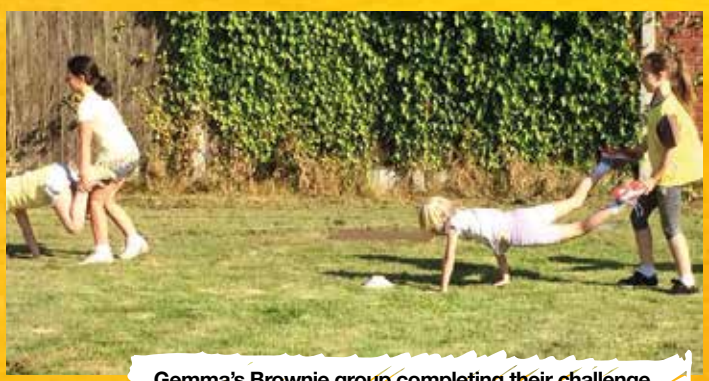
Brownie Leader, Gemma Roberts, paired her Brownies background with her passion for raising awareness of CF, and created two badges to help raise funds for the Trust.

Gemma created the 'Cystic Fibrosis Challenge' badge first, which Brownies can earn by completing a number of CF-related challenges.

Gemma also created a thank you badge, from which all profits will be donated to the Trust.

Gemma created the 'Cystic Fibrosis Challenge' badge as part of her Queen's Guide Award.

"A few years ago, a family friend was diagnosed with CF at the age of 15, and when her brothers were tested to see if they were carriers, they found out that her oldest brother also had the condition. I didn't know much about CF, and it was a massive shock to realise just how common it is in the UK, and to see just how big an impact it had upon my friend's life. The Trust works hard to support those with CF, like my friend, and I decided that I would do what I could to support them." - Gemma



Gemma's Brownie group completing their challenge

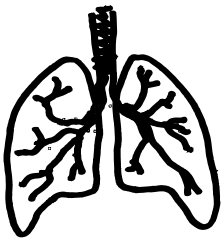
The challenge

There are five sections to the challenge badge:

- **What is cystic fibrosis?** – these challenges encourage Brownies to learn more about cystic fibrosis
- **Exercise** – this section teaches the importance of exercise for people with cystic fibrosis
- **Diet** – these challenges show how people with CF can have different nutrition needs from people without the condition
- **Raise awareness** – this section is all about raising the profile of cystic fibrosis
- **Fundraising** – these challenges help to raise funds for the Trust

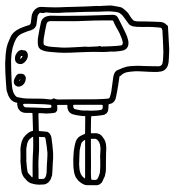
Gemma's Brownies unit earned their 'Cystic Fibrosis Challenge' badge by completing an obstacle course as many times as possible in half an hour, raising over £420 for the Trust!

Why not try some of Gemma's challenges yourself?

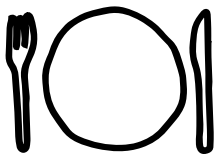


Challenge: Make a model of the lungs

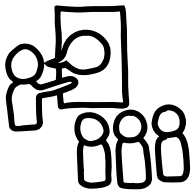
Challenge: Learn how to wash your hands properly to get rid of all the germs



Challenge: Try making a recipe from www.chef4cf.com/recipes



Challenge: Create a diet sheet to help people understand what foods people with CF should eat



Challenge: Take a poster into school for show and tell and teach your friends all about cystic fibrosis

Challenge: Hold a sponsored event such as a sponsored silence, run, abseil or dance-a-thon

Gemma's Brownie group

Sending packaging packing

Part-time marine biologist Robin Masefield decided to make his passion for the environment his business, when he and wife Chloe opened 'Natural Weigh', Wales's first zero-waste shop, in Crickhowell.

The environment has long been an important issue for Robin, made stronger still by living with cystic fibrosis.

"I loved studying Marine Biology and it was whilst studying and in my career that I really began to develop my love for the environment. I became increasingly aware of the damage we are causing to it through so many of our activities, including plastic pollution.

"I definitely feel that CF has influenced my environmental concerns in more recent years. I feel that we are, in part at least, a product of our surroundings. That's not to say CF is caused by our environment but that environmental factors (air quality for example) play an important factor in our health."

"I definitely feel that CF has influenced my environmental concerns"

– Robin

"The problem of litter and plastic pollution is so widespread. We are consuming microplastics in the food we eat and the water we drink on a daily basis!"

Robin and Chloe felt they could influence the problem of packaging directly. "Sell food, cleaning and lifestyle products without the unnecessary plastic packaging and we directly reduce the amount of waste being produced."

A place for plastics

Robin says: "I would certainly like to see less packaging with my medication and at clinic where feasible. That said, I do feel that plastic is an incredible material when used appropriately and should be a highly-valued resource… In clinic it ensures the risk of cross-contamination and infection is minimal."

Robin's advice for other people with CF considering running a business? "Have a plan for when you need some time out to take care of yourself and make sure you have someone you can rely on to help. My wife is amazing and always makes sure I'm looking after myself. It's a lot of work, we do long days and often spend time in the evening doing admin, so anything to make it easier is a must. If it's your passion, don't let CF stop you, embrace the challenges!"


Robin and Chloe, in their shop.

"If it's your passion, don't let CF stop you, embrace the challenges!."

– Robin

Do you have a business or a hobby you'd like to share with us? Email magazine@cysticfibrosis.org.uk.

Building the foundations for a Life Unlimited

As we embark on our new research strategy, it's a good time to take a step back and look at what your support has helped achieve since we published our first strategy in 2013.

In lots of ways making progress in research is like building a house - you have to lay the foundations first and gradually build on them bit by bit. In a similar way, it can take years to understand the difference a piece of funding we provided has made to improving our knowledge of cystic fibrosis. Here are some examples of where we've got to so far.

Preparing the ground

In 2013 we set up two new ways of funding CF research – Strategic Research Centres (SRCs) and Venture and Innovation Awards (VIAs). We've committed to funding 14 SRCs so far and more than 50 VIAs. Within our SRCs more than 96 investigators, from psychologists to geneticists to microbiologists, from over 14 different countries have put their heads together to work on a number of vital areas of CF research. Because they're based all around the world, whatever time you're reading this article, there's someone working on a piece of CF research right now!

VIAs have brought together researchers in a different way, allowing us to co-fund research into a diverse range of problems. Every £1 we spent we leveraged almost £4 from other organisations (turn to page 10 to find out more).

We've also been able to encourage younger researchers into the field of CF – so hopefully they'll be there for many years to come. 41 early career researchers are working on our SRCs, where they're building their skills, knowledge and research networks. They've also been enthusiastic in explaining their research to the CF community and the wider public, from talking at CF's Got Talent! at the UK Cystic Fibrosis Conference to presenting at science festivals up and down the country!

Research for today and tomorrow

When we were putting together our last research strategy you told us that it was important to have a balanced portfolio of research projects that would improve symptoms ("research for today") as well as work to stop the condition in its tracks ("research for tomorrow").

So far, we've funded SRCs on a range of topics that affect people with CF day-to-day, from lung infections such as Pseudomonas and NTM to complications like diabetes and arthropathy.

In working to stop CF we're funding gene editing to repair mistakes in the damaged CF gene, studying the structure and function of the CFTR protein in more detail and looking at alternative drug approaches to modifying the CFTR protein itself.

Plugging the gaps

With so much going on, some researchers may not be aware that their expertise could help us understand more about a particular area of cystic fibrosis. Sometimes the Trust needs to make introductions, which is where our research sandpits come in.

With some matchmaking help from the Trust, researchers met to understand some of the gut complications of CF and share their expertise. Teams brought together in the 'gut sandpit' went on to start their own SRCs in this vital research area.

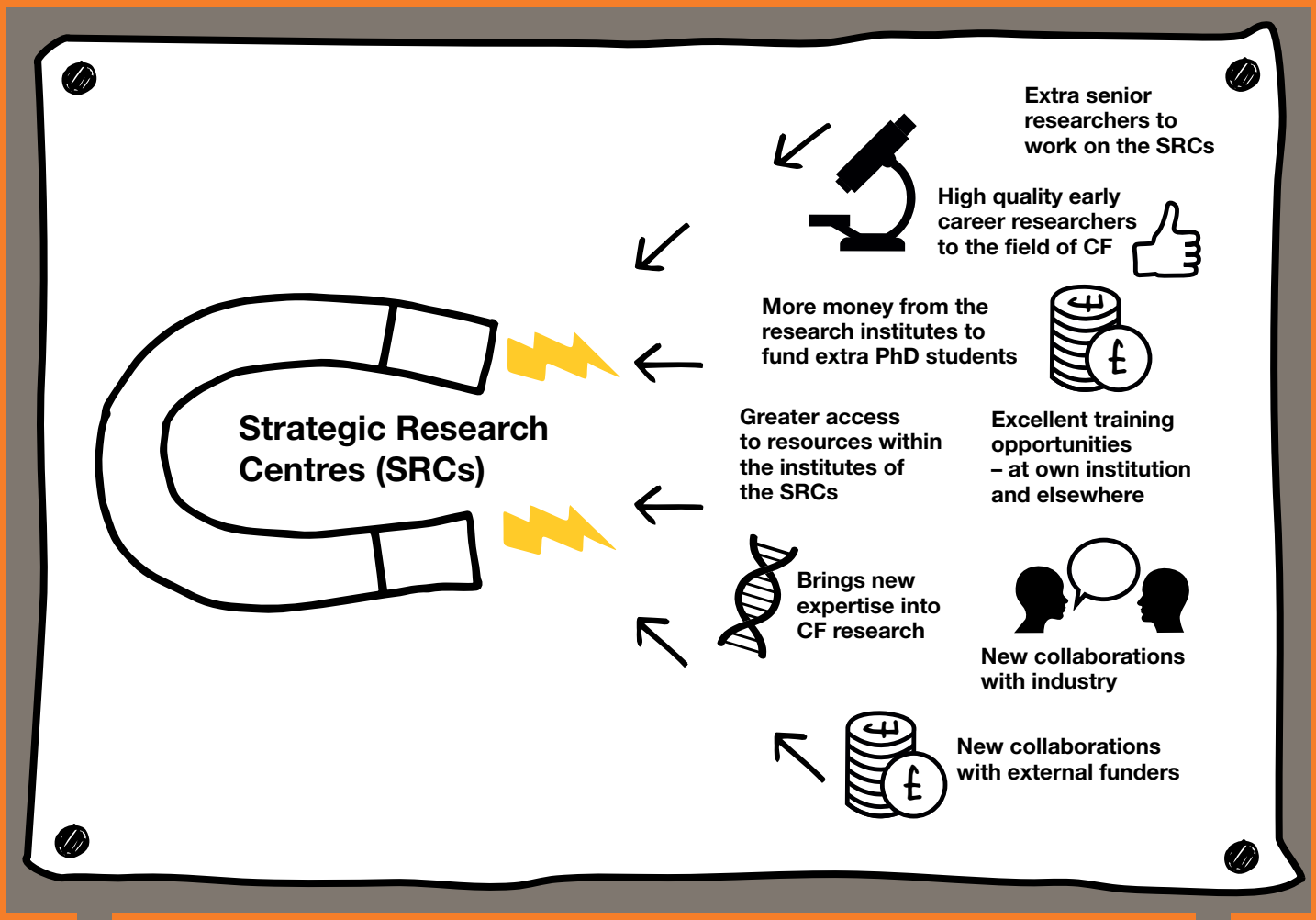
Another brick in the wall

The impact of the last research strategy is already starting to show. For example, studies in the first Pseudomonas SRC showed that its sub-strains act in slightly different ways. Researchers working on the SRC, led by Professor Jane Davies at Imperial College London, started to collect and store samples of Pseudomonas and now have a fantastic (albeit grizzly!) resource of different strains, grown from samples from people with CF who came into the Royal Brompton clinic.

In the new, recently-funded 'PAPA' SRC, researchers will be using these samples to understand and treat the bug on a strain-by-strain basis. Without that initial project, the new SRC research wouldn't be taking place.

Our new research strategy

The research we funded over the last five years has shown us which direction we need to take for the future. We're aiming towards delivering personalised treatments for people with cystic fibrosis. **Visit cysticfibrosis.org.uk/researchstrategy to find out more.**



If you build it...

There were some great 'knock-on effects' that we didn't anticipate when we published our strategy. For example, the SRCs attracted more expertise, external funds and access to resources than we, or the researchers working on them, expected.

Jade talks trials: from drawing board to studio

Jade Ashton is 22, has CF and has just completed her Masters in Biomedical Sciences at the University of Southampton. She is also a member of our Youth Advisory Group (YAG) and recently helped to develop a clinical trials video for young people.

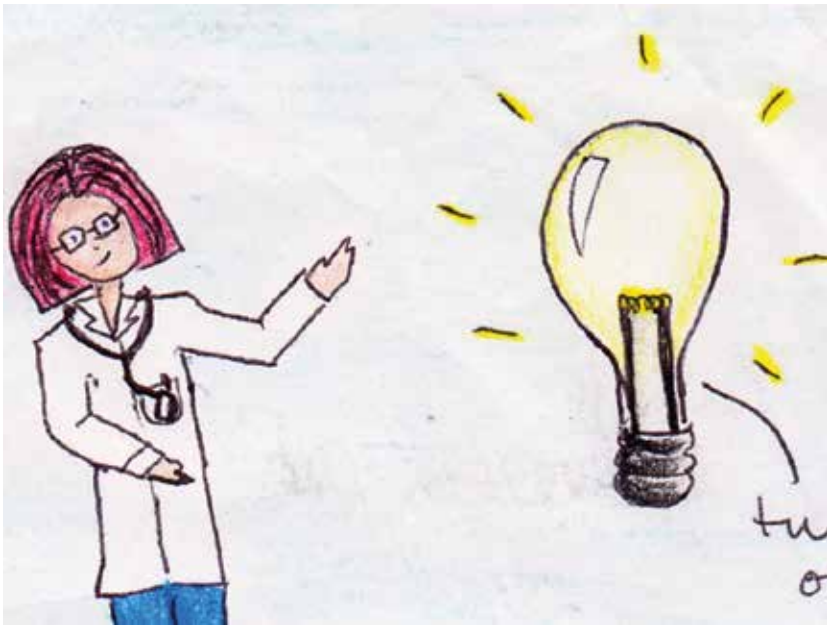
Having CF has always made me want to learn more about the human body. I've never hidden my passion for increasing awareness of CF science and research, and participation in clinical trials plays a big part in that. Holly-Rae Smith, the Trust's Youth Empowerment Officer, is really great at assigning projects to members of YAG that she knows will suit us, so when the clinical trials video came up she knew I'd be up to the task!

With access to Orkambi being debated in Parliament, information from clinical trials is crucial in informing decisions. And trials aren't just limited to treatments - I'm currently taking part in a trial that's looking at the reaction of blood vessels and glucose levels in people with CF taking part in intense exercise, so trials can also improve our understanding of the condition too.

I wanted to help make this video to answer questions, dispel myths and reduce any fear surrounding taking part in clinical trials.

Jade in the recording studio.

I used emojis and bright colours to try and make the topic friendly and engaging, and used imagery that everyone could recognise as 'sciency', like the conical flask and pills.



"It has been such a pleasure to see this animation develop from Jade's first email. It's a fantastic example of what can be achieved when the Trust collaborates on an idea led by a young person living with cystic fibrosis. A huge thank you to Jade for her hard work, even while writing a dissertation, and to the Queen's Trust for funding the project."

Holly-Rae Smith,
Youth Empowerment Officer.

I thought having a smiling doctor as a narrator would help to get the point across that clinical trials are safe and accessible. It was lovely, if a little surreal, having the doctor I drew transformed into a cartoon version of me in the animation!

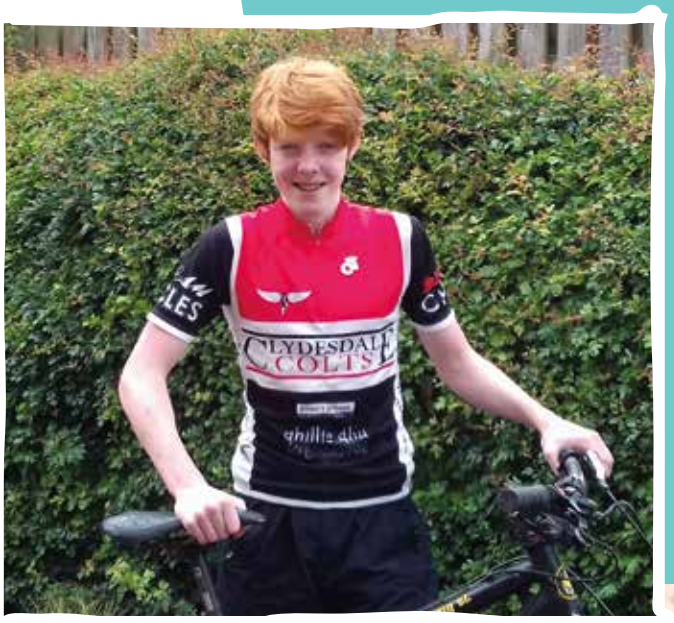
I was a bit apprehensive sending off my initial ideas to the Trust as I was worried I'd gotten a bit carried away but thankfully everyone really liked it. When I was asked whether I would like to do the voiceover for my script as well, I was naturally very keen.

I was kept really involved in the production process and my opinion was often sought for the drafted animations. It was great to work with talented animators that really brought my ideas to life, and even though the finished product turned out quite different due to script changes and animation logistics, they definitely stayed true to my vision.

Don't forget to take a look at the Trust's Trials Tracker (cysticfibrosis.org.uk/trialstracker) to see what you can get involved in, or ask your CF team. Could you change the future of CF? Together, I think we could.

Visit cysticfibrosis.org.uk/youngpeopletrials

A wheelie fun way to keep fit

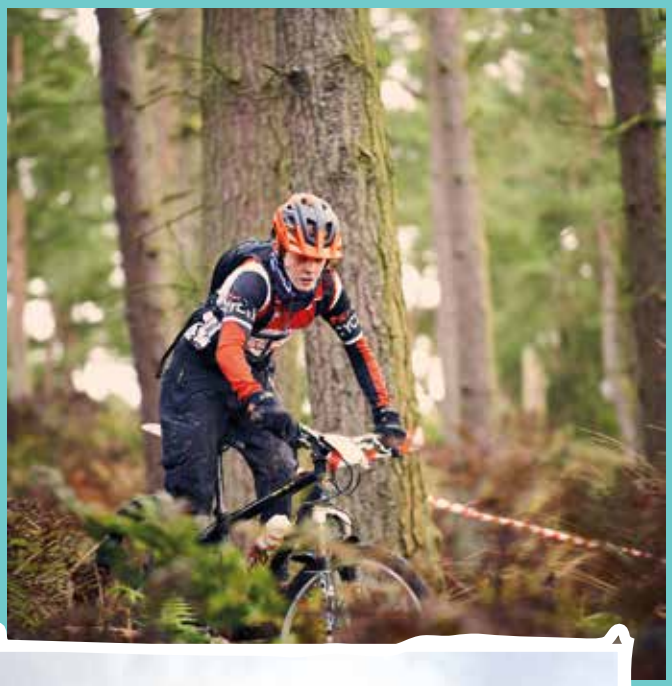


In previous issues we've shared exercises people with CF can do at home, but what about when the great outdoors beckons? Here, 16-year-old Cameron from Lanarkshire shares his passion and focus: biking.

I am a keen mountain biker. I try to go out four times a week and occasionally substitute physio sessions for a bike ride as I feel it has a good effect on my lungs.

Cycling started becoming a passion around age 14. Over the past year I have progressed, got fitter and, most importantly, had more fun on the bike than ever before. I rarely feel demotivated to go out, even if the weather isn't the best. If I am out and things go south (rain comes on or I get cold and wet), I always remind myself that it will be over in an hour or so.

If my CF is playing up I get frustrated as I can't go out as much. I always stay positive though - it will clear up and I'll be back at it soon! My biggest achievement is completing the Strathpuffer, a 24-hour mountain bike race in the Highlands, in the middle of winter, which I completed as part of a team of four.



"Anyone can start cycling, no matter their age or fitness level, because you can go as fast or as slow as you like and then progress."

I try to go out enough to keep me fit and make sure I'm always improving, however even twice a week is enough to make improvements if you are looking to start. Anyone can start cycling, no matter their age or fitness level, because you can go as fast or as slow as you like and then progress.

Another way to get into cycling is with friends or a club. I find going out with people is more enjoyable - you can also encourage each other to do jumps or push each other further. However, I feel I get better and fitter going out myself because I can focus more, and still have a good time. Anytime on the bike is a good time!

I will continue mountain biking for the rest of my life, it only has a positive impact on my CF and my life. I'm always thinking about my next ride and I live for the sport: without my bike I don't know where I'd be.

Inspired by Cameron and interested in taking on a cycling challenge? See what we've got in store at cysticfibrosis.org.uk/cycling

Days in the life

When it comes to talking about CF and the issues that matter, there's no substitute for personal experience – you're the experts!

In her work, 24-year-old blogger/vlogger (and dog walker) Kate Eveling casts her unique eye on the world, from clinic visits to burning issues to holiday diaries. For Days in the Life, Kate shares her creative process with us.

First I start by writing down an idea for a video and then I create a shot list!

I then set up the equipment like a tripod and a camera (essential!)

I'm a one-woman band as I direct, produce and star in all my videos.

4

5

6

Want to see the end results? Head over to Kate's YouTube channel to check out some of her amazing films: www.youtube.com/KateEveling

cysticfibrosis.org.uk/giftsinwills

Cystic Fibrosis worth a minute of your time

You don't have to be magic to do something extraordinary

Thank you to those extraordinary supporters who have included a gift in their will to support our future work.

Gifts in wills help us know we can continue fighting long into the future.

giftsinwills@cysticfibrosis.org.uk

Fighting for a Life Unlimited