

Cystic Fibrosis Trust

Impact Report 2019/20



Fighting for a
Life Unlimited

What is cystic fibrosis?

Cystic fibrosis (CF) is a devastating condition that affects over 10,600 people in the UK. It's caused by a faulty gene that affects the movement of salt and water between cells, and fills the lungs with thick mucus, making it hard to breathe and clear harmful bacteria. The mucus also stops the digestive system from breaking down food effectively and makes people with CF susceptible to catching dangerous bacteria, meaning that they can never meet each other in person.

While there is no cure, there are treatments, but these carry a heavy burden. Most people with the condition will have to undertake hours of physiotherapy and inhaled treatments every day, and consume handfuls of tablets to digest their food. Lung infections cause frequent hospitalisation and other complications affect the bones, liver, reproductive system and other parts of the body. A double-lung transplant is often the last resort for people with cystic fibrosis. While babies born with the condition today are expected to live much longer than they were years ago, thanks to new treatments and better care and understanding of the condition, the median predicted age of survival is only 49 years of age. Sadly, the average age at death is currently just 31 years.

What are we doing about it?

The Cystic Fibrosis Trust is fighting for a life unlimited for everyone affected by cystic fibrosis. That means funding ground-breaking research, campaigning hard on the issues that matter, providing vital information, support and emergency welfare grants when needed, as well as promoting the highest quality of clinical care.

In this report you'll hear about everything we've been doing this past financial year and find out how your support has impacted people with CF, whether you've donated or fundraised to support our work or lent your voice on the issues that matter.

Without your support, we wouldn't be able to do the work we do. Thanks to you, we are moving closer every day to a world where people with CF can live their lives unlimited by this cruel condition.

*Thank you from everyone
at the Trust.*





Contents

Funding ground-breaking research	4–5
Clinical Trials Accelerator Platform	6
Quality of care	7
Campaigning hard	8–9
UK CF Registry	10
Providing information and support	11–12
Looking ahead	13
Our extraordinary supporters	14–15
Accounts	16–17
Sixty Five Roses Club	18
Thank yous	19

(On the cover: the Payne family who took part in our Superheroes campaign)

“Thanks to the commitment, resilience and dedication of the community over many years, this last year – and past few months – have seen incredible progress in access to life-saving drugs for people with cystic fibrosis. We know that there is more to do and will continue to fight for a life unlimited for all those affected by the condition. In the past year, the Trust has continued to support a dynamic research portfolio and clinical developments that give the hope of further new treatments. We have also worked hard to offer trusted information and vital support to those who need us most.

“With the unprecedented upheaval of the coronavirus (COVID-19) pandemic, the year ahead will bring new and unexpected challenges. We will continue to support our community, and will adapt, improve and always remain focused on making a difference for everyone living with cystic fibrosis.”

– David Ramsden, Chief Executive

Funding ground-breaking research

We are the biggest charity funder of research specifically for the benefit of people with CF in the UK. New research is vital for discovering the next transformational breakthrough that will help deliver our vision of a life unlimited and improve the lives of people with CF forever.

What we achieved this year



30th anniversary of the CF gene discovery



2 new Strategic Research Centres (SRCs) awarded



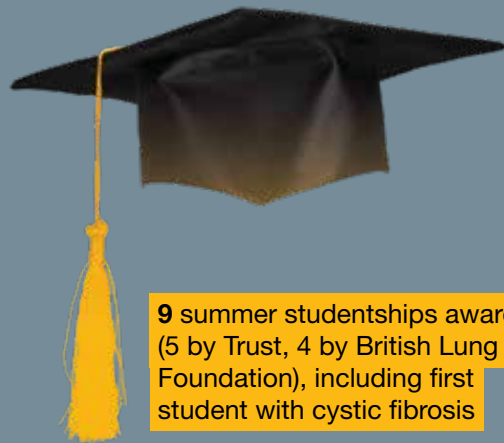
£500,000 spent on Venture and Innovation Awards (VIAs) and **£4 million** leveraged in external funds



82% excellence-rating at our two-day UK CF conference, with **350** delegates and **260** online participants



5 internationally-based researchers presenting at CF's Got Talent (at UKCFC)



9 summer studentships awarded (5 by Trust, 4 by British Lung Foundation), including first student with cystic fibrosis

Find out more at cysticfibrosis.org.uk/trustresearch

Innovation Hub

The UK Cystic Fibrosis Innovation Hub is a ground-breaking partnership between the Cystic Fibrosis Trust and the University of Cambridge, bringing together the UK's leading experts in lung health to prevent lung damage in cystic fibrosis.

Together, the Innovation Hub team aims to make rapid advances towards new treatment tools, drugs and approaches for CF, specifically:

- developing better antibiotics and anti-inflammatories;
- predicting when exacerbations are likely to happen and what causes them; and
- looking at ways to repair existing lung damage.

Researchers within the Innovation Hub are focusing on developing new antibiotics to treat *Pseudomonas aeruginosa* and *Mycobacterium abscessus* infections, which have devastating effects in people with cystic fibrosis.

Our aim is to raise £5 million over five years, which the University of Cambridge has committed to match pound for pound. Up to the end of March 2020, **£1.8 million** has been kindly donated and pledged for this programme.

We are so very grateful for the support shown to this project, and would especially like to thank The Robert Luff Foundation and AJN Steelstock, along with a number of individual donors, trusts and companies.

Find out the latest updates at
cysticfibrosis.org.uk/innovationhub



"I think the Innovation Hub research is absolutely extraordinary. For me, a better treatment could have meant spending less time in hospital, having fewer side effects to the IV antibiotics and more time at home with the people I love."

– Mehro-Nissa, who has CF, was recently diagnosed with *M. abscessus* infection

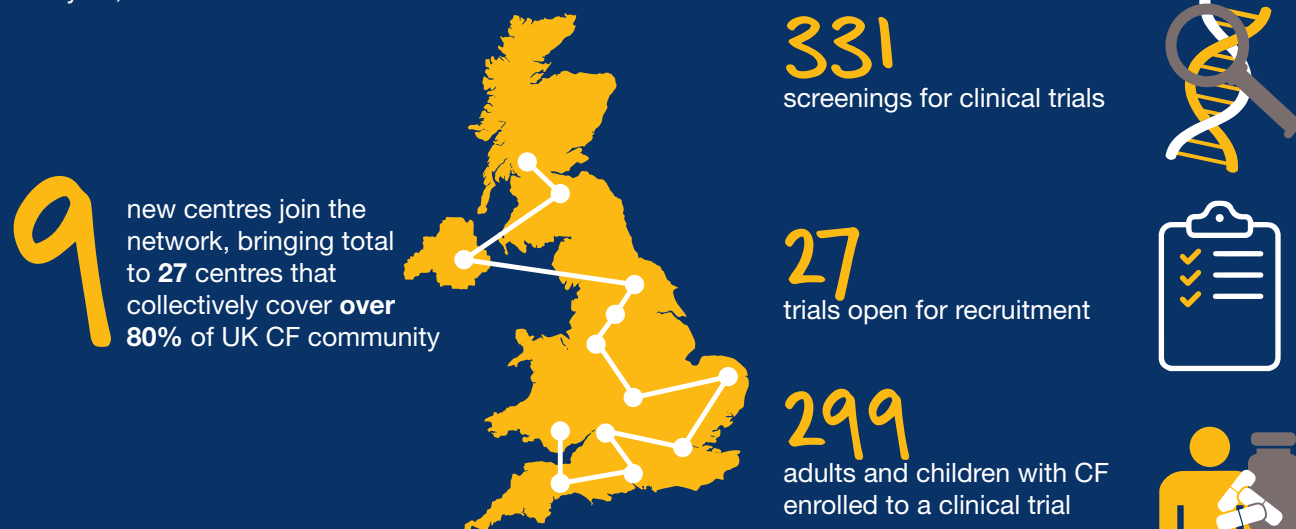


Mehro-Nissa

Clinical Trials Accelerator Platform

The Clinical Trials Accelerator Platform is a UK-wide initiative bringing together CF centres to increase access to CF clinical trials. By creating a robust infrastructure for clinical trial delivery, we are ensuring the UK is speeding up the development of breakthrough CF therapies.

This year, the Trials Accelerator has seen:



“The Trials Accelerator has changed the entire landscape for CF clinical trials at our site. Our portfolio has grown from strength to strength, and patients are so much more aware of what is on offer.”

– Jessica Longmate, Trial Coordinator, Nottingham University Hospital

Thanks to funding from our partnership with the Cystic Fibrosis Foundation, we can extend and expand the Trials Accelerator, including:

- **3-year** extension of current Trial Coordinator posts;
- **6** new Trials Coordinators to support delivery of early- phase CF clinical trials; and
- a Lung Clearance Index (LCI) training package for participating centres.

We continue to provide representatives from the CF community with the opportunity to feedback on how to make trials more accessible and participant-friendly, with **over 50** participants forming our Patient and Public Involvement group.

Visit cysticfibrosis.org.uk/clinicaltrials to find out more.

Quality of care

We are committed to improving the quality of care in CF centres across the UK, ensuring healthcare is safe, timely, effective, efficient, accessible and people-centred.

This year, we launched:

- **The Patient Reported Experience Measures Survey (PREMs)**, our UK-wide patient experience survey that gives participating CF centres insights into what their patients and families think about the health care they receive and what areas to improve.

The paediatric survey received **700 responses**, engagement from **75%** of all UK CF centres. The adult survey will launch in 2020-21.

“I have received a great service from this team. From the time our child was born they have been there every step of the way, not just for our child but for me when I have been struggling. Amazing team!”

– Comment captured from paediatric PREMs

- **UK Staffing tool**, providing CF centres with a yearly snapshot of staffing levels and resources in participating centres, compared to UK averages.

We engaged with **78% of centres**. Centre-level and UK reports will be available in 2020-21.

Find out more at
cysticfibrosis.org.uk/qualityimprovement



Campaigning hard

We campaign hard to ensure the voice of the CF community is heard loud and clear.

At the time of writing, we have received the fantastic news that 'Kaftrio', the first triple combination therapy to treat the root cause of CF, has been officially licenced for use by the European Commission. This follows deals made in all four UK nations, bringing hope to thousands of eligible people with cystic fibrosis.

This news is a big step in a long campaign for access to life-saving drugs, built on the years fought alongside the CF community for access to Orkambi and Symkevi. We recap some of the key moments in 2019 that led to this monumental breakthrough, which wouldn't have been achieved without the strength and resilience of all those who supported this campaign:

April

- Worked with parliamentarians across the four UK nations to table parliamentary questions, including a PMQ in Westminster.

May

- Handed a bouquet of 65 yellow roses to 10 Downing Street, with a letter from **77 MPs** calling for action.

June

- Hosted an online focus group for people with CF to brief Paul Scully MP on their experiences ahead of a Westminster Hall debate, which saw **41 MPs** speaking on behalf of people affected by cystic fibrosis.

August

- **SMC announces it is not able to recommend Orkambi and Symkevi for routine use by NHS Scotland due to price concerns, but discussions continue between Vertex and the Scottish Government.**



September

- **The Scottish Government reaches an agreement with Vertex, supported by the UK CF Registry, announcing a five-year deal for access to Orkambi and Symkevi.**

October

- **England reaches a two-year managed access agreement, giving eligible people with CF access to Orkambi, Symkevi and Kalydeco.**

November

- **Northern Ireland and Wales formalise agreements for access to Orkambi and Symkevi.**

“I don’t think there’s a word that fully describes all the emotions I was feeling when the announcements were made. I was so excited and much more hopeful for the future. I had been suffering with infections that were substantially lowering my lung function but, since starting on Symkevi in December 2019, it’s given me the stability and reassurance I needed, and gives me hope for what lies ahead. I’m so grateful to live in a time where CF treatments are developing so quickly and effectively, and where getting older isn’t so much of a concern.” – Cerys

“After years of fighting in this campaign, it was a shock that it was finally over... a shock that turned into happy tears, knowing I could feel optimistic about Cerys’ future. The Trust continues to be a strength to me, and I know they are fighting for me at times when I cannot.” – Catherine, Cerys’ mum



Cerys and Catherine

While we celebrate this incredible milestone, we know that there is more to do, and we will not stop until everyone with CF across the UK has access to life-saving drugs.

Visit cysticfibrosis.org.uk/campaigns to find out more about our campaign work.

UK CF Registry

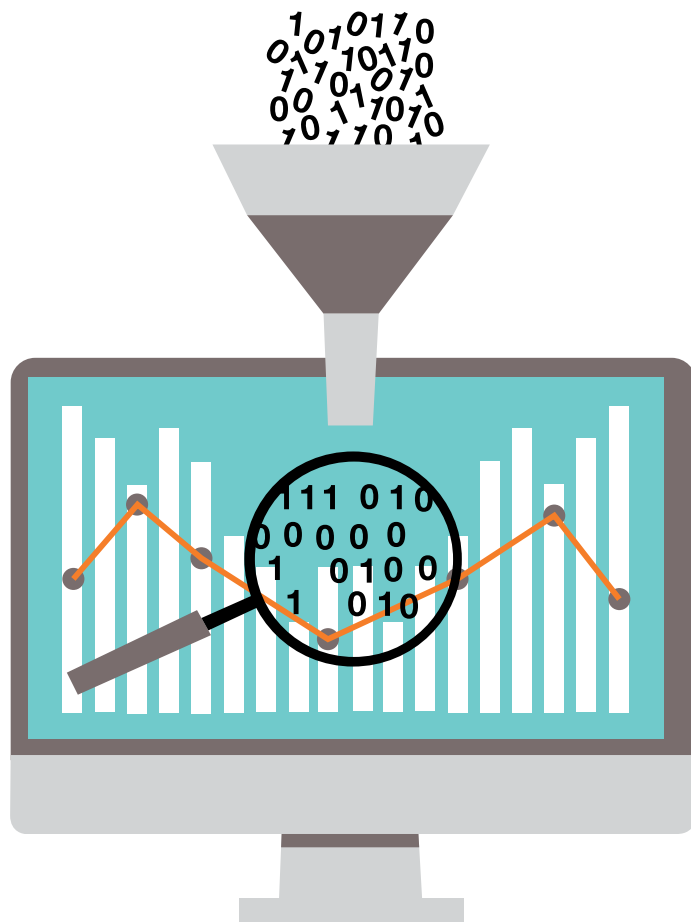
The UK CF Registry is a world-class database that records data on over 99% of people with CF in the UK, who have consented to their data being submitted by their care team. The Registry uses data to improve the health of people with CF through research, guide quality improvement at care centres, and monitor the safety of new drugs.

The impact of the Registry

Following interim access agreements in all four nations, the UK CF Registry will be playing a pivotal role in monitoring the effectiveness of Orkambi and Symkevi, with monitoring activities recently extended to include the triple combination therapy, Kaftrio.

The Registry team will report on routine and vital real-world data, helping public health bodies in the UK to evaluate the efficiency of current and future therapies.

The information collected as part of these access schemes will be fundamental in evaluating the impact of future therapies, helping to secure ongoing access to these life-saving drugs in the future.



Read the latest at cysticfibrosis.org.uk/impactofregistry or find out more about the Registry at cysticfibrosis.org.uk/registry

Providing vital information and support

Cystic fibrosis is a complex condition that makes it harder to live a healthy, happy and productive life. We provide trusted, accessible and reliable information and support in all areas, helping the CF community get the help they need each and every day.

375

people supported by our Specialist Welfare Advisor, helping people access at least **£1.43 million** in benefits

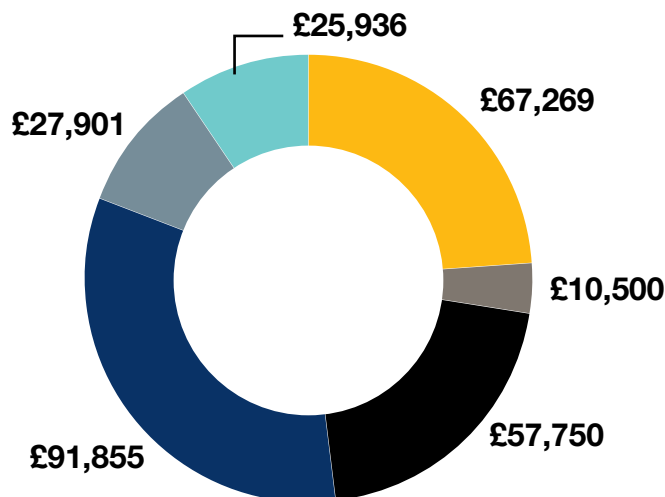


This year we awarded **1,050 welfare grants, totalling £281,212**



22

young people affected by CF in our Youth Advisory Group, helping to shape the direction of our youth work



Emergency Transplant Funeral
Health and wellbeing Holiday Homecare

117

engagements through our Building Brighter Future workshops for young people with CF



3,600

enquiries received to our Helpline, **40%** relating to financial concerns or support

Find out more about the information and support we provide at **cysticfibrosis.org.uk/support**

Tyler's story

Applying for benefits when you have an 'invisible' condition can be challenging – especially when assessors underestimate just how much of an impact it can have on your life.

Tyler was receiving Disability Living Allowance (DLA) but was informed he had to claim Personal Independence Payment (PIP) around his 19th birthday. When he received a decision, Tyler found he had been refused points on his ability to “manage therapy or monitor a health condition” – an area of PIP that poses significant issues for people with cystic fibrosis. This meant he didn't have enough points to qualify for PIP and so lost his benefits completely.

Tyler and his mum Gaynor reached out to our Welfare and Rights Advisor, Sangeeta, who worked with the family to appeal this decision. She guided them through a 'First-tier Tribunal' and, after still not receiving enough points for PIP, advised him further to appeal to an 'Upper Tribunal'.

At Tyler's next hearing, the Department for Work and Pensions agreed there had been errors of law in the decisions made about his PIP claim. At the Upper Tribunal, the judge also found that there had been an error of law. The original Tribunal's decision was therefore set aside (no longer stands/valid).

Following the Upper Tribunal decision in January, where the original decision from 2017 was 'set aside', the appeal was heard again by a First-tier Tribunal. Sangeeta represented at a telephone appeal hearing, along with Tyler, Gaynor and a social worker. Tyler won his case and his PIP award will be paid from February 2016 until August 2022. Tyler and Gaynor's persistence in taking Tyler's case higher up the courts will have a huge impact on people with CF applying for PIP, now and in the future.



“We would never have been able to do this without the help and support from Sangeeta and the Trust, throughout this whole process.

“Unfortunately, the people that do the assessments for PIP have very little knowledge of CF and how it impacts people's lives both physically and mentally.”

– Gaynor, Tyler's mum

Read the whole story at
cysticfibrosis.org.uk/burdenofcare

Looking ahead

Coronavirus (COVID-19)

This financial year came to an end under particularly difficult circumstances, especially for those affected by cystic fibrosis. At the time of writing the COVID-19 crisis is still unfolding. With challenge events being cancelled, our fundraising income has significantly fallen and future plans may be forced to change. However, we will continue to keep up with the rapidly evolving situation to do all we can to support people with CF and their families through this current crisis and beyond. We will also adapt to find new ways to fundraise in the face of challenges posed by COVID-19.

Securing access to Kaftrio

At the time of writing, the European Commission has officially licenced the triple combination therapy 'Kaftrio' for use. Leading up to this announcement, governments across the UK have each brokered a deal so that thousands of eligible people with CF can gain access at the earliest opportunity. Moving forward, we will work to ensure that the roll-out of Kaftrio extends to everyone who could benefit from it, across all nations. We will also redouble our efforts on the significant numbers of those within our community who will not be eligible for this treatment, so that everyone with CF can have a future to look forward to.

Marc Cotterill, one of the many who can benefit from Kaftrio

Promoting our research and clinical advancements

We will continue to focus on securing access to the best possible medicines for all people with CF, including those who won't benefit from the 'CFTR' modulators currently available, as well as securing funding from external sources and using novel approaches to tackle important issues. Through building on our Trials Accelerator platform, we will improve access to clinical trials and the breakthrough of new, life-changing treatments.

Championing CF and the challenges it brings

We will champion the voice of the CF community, making sure they are heard on the issues that matter and represented in how clinical care is designed and improved. We will continue to improve our understanding of the current and future needs of the community, increase our focus on ensuring all our services and resources are accessible and representative, and seek to reach all those most in need of support.



Our extraordinary supporters

This past year we have made huge developments in our fight for a life unlimited and created real change for all those affected by the condition. We couldn't have done this without the support of our incredible Community and Events fundraisers.

3,000 event participants



1,512 community events



546 walkers across our Great Strides™ series



310 Wear Yellow Day events

123 London Marathon runners



"The money you raise could pay for the research that finds the cure to give our son a life unlimited."

– the Payne Family

Our corporate partners are invaluable to us – their expertise, knowledge and fundraising skills all combine to help us deliver a life unlimited for people with cystic fibrosis.

Outdoor advertising company Clear Channel have been our corporate partner for over four years and provided us with advertising space across the UK, giving us a voice that we could not otherwise afford. With Clear Channel's support, we ran adverts on billboards and bus stops throughout the year, raising awareness of the impact of CF and promoting key events such as CF Week.

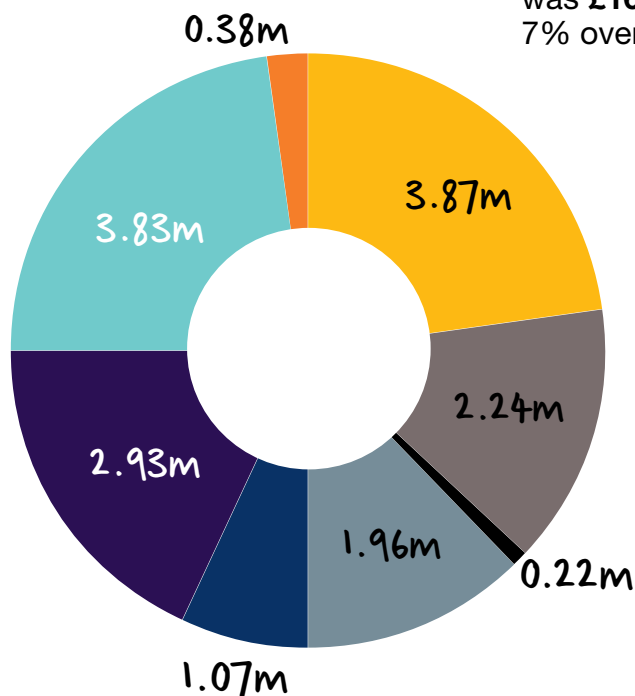
“We are proud of our on-going partnership with the Cystic Fibrosis Trust and continue to leverage our public medium as a platform for good to support the Trust’s valuable and vital work and help make a difference to people with CF across the UK.”

– Richard Bon, Joint Managing Director,
Clear Channel



Where our money comes from

Our income from normal activities was **£16.5 million**, a decrease of 7% over the previous year.



Branches, groups and community

Regular and individual giving and appeals

Corporate partnerships

Trusts

Cystic Fibrosis Services Limited*

Legacies

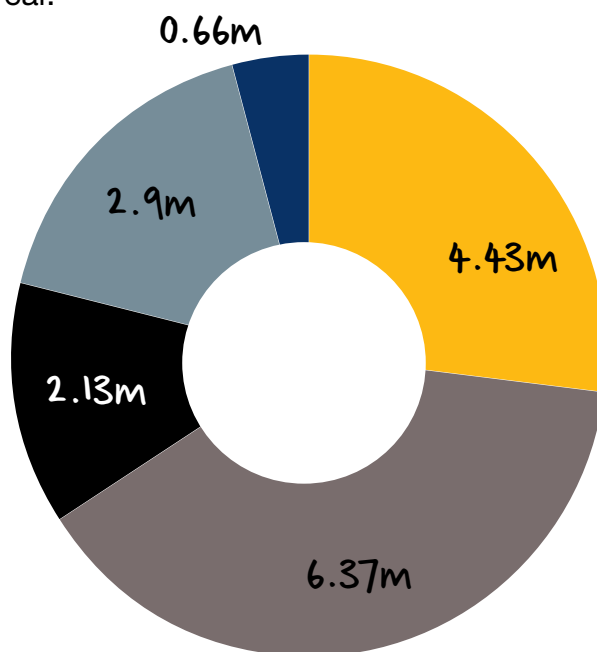
Donated goods and services

Other income

*The subsidiary's activities include medical services, information and data system support and the merchandising of marketing materials.

How our money is spent

Our total expenditure for the year was **£16.5 million**, a 5% increase compared to the previous year.



- Research
- Information, advice and support
- Clinical care
- Costs of raising funds
- Cystic Fibrosis Services Limited*

Full details of our accounts can be found in our Annual Report and Financial Statements at cysticfibrosis.org.uk/annualreview

*The subsidiary's activities include medical services, information and data system support and the merchandising of marketing materials.

Sixty Five Roses Club

Join our Sixty-Five Roses Club for exclusive access to a quarterly newsletter, bespoke events and the opportunity to meet likeminded individuals helping us fight for a life unlimited.

A generous pledge to gift a minimum of £1,000 a year, for a minimum of three years, will guarantee your membership to the Sixty-Five Roses Club. These donations will help to fund vital projects.

Email sixtyfive@cysticfibrosis.org.uk for more information on joining.

"Every member of the Sixty-Five Roses club has helped to create a brighter future for people living with cystic fibrosis. It is more than a philanthropic giving club; it is a community of kind people who enable and support the life-changing work that the Trust does. I am grateful to all the members, and proud to be the patron of the Sixty Five Roses."

– Rob Law MBE, creator of the Trunki children's suitcase and Sixty Five Roses Club Patron



Rob Law MBE

Thank you

We would like to thank all of the trusts and organisations that we work with, some of whom are mentioned on this page.

Collaborating globally is vital to ensure we are providing the best possible support to the CF community. We engage internationally in a number of ways and would particularly like to acknowledge partnership funding from the Cystic Fibrosis Foundation.

Special thanks

An extra special thank you to our incredible fundraisers, challenge and event participants, and volunteers and donors, including our branches and fundraising committees who are so very generous with their time and support.

Those named on this page are just some of our supporters, we would also like to acknowledge those who wish to remain anonymous. We simply couldn't do anything we do without your help!



Individuals and families

- June Bedford ■ Boyden Family ■ Mrs N Carr
- Mrs Sally Khalil ■ Dr J Winn ■ Jack Burnham
- Chandru Kishinani, former EMS Limited

Corporates

- AJN Steelstock ■ David Wilson Homes South Midlands
- LendInvest ■ Bird & Bird ■ Clear Channel
- Goodstuff Communications

Trusts

- Alan Miller Charitable Trust ■ BBC Children in Need
- Joseph Levy Foundation ■ Rosetrees Trust
- Sir Samuel Scott of Yews Trust ■ Sport England
- The Gay and Keith Talbot Trust
- The Morrisons Foundation ■ The Queen's Trust
- The Revere Charitable Trust
- Robert Luff Foundation ■ The Sinclair Charity

Gifts in Wills

We extend a warm appreciation to all generous supporters who recently died and kindly remembered those with CF with a gift in their Will. Gifts in Wills help ensure our future work and allow us to continue fighting CF until we beat it for good. The family and loved ones of the following estate agreed to acknowledgement of the gift kindly received.

- Dr Hans-Hilmar Spechter

Online Book of Remembrance

Our Online Book of Remembrance remembers all those who kindly thought of us in such an extraordinary way to shape the future.

You can view the Online Book of Remembrance on our website: cysticfibrosis.org.uk/bookofremembrance

Cystic Fibrosis Trust

Find us online

cysticfibrosis.org.uk

 [@cftrust](https://twitter.com/cftrust)

 [Cystic Fibrosis Trust](https://www.facebook.com/CysticFibrosisTrust)

 [cftrust](https://www.youtube.com/c/cftrust)

 [@cftrustuk](https://www.instagram.com/cftrustuk)

 [linkedin.com/company/cystic-fibrosis-trust](https://www.linkedin.com/company/cystic-fibrosis-trust)