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Cystic Fibrosis why we're here

Making a step-change:
Four programmes building a personalised future for people with cystic fibrosis

Fighting for a Life Unlimited

When the Cystic Fibrosis Trust was founded more than 50 years ago, a child born with cystic fibrosis (CF) was not expected to reach their fifth birthday. Today, as a result of step-by-step improvements in care over these last five decades, they stand a good chance of reaching their fiftieth.

Yet we are still a long way from beating this cruel condition. Too many people born with CF will not live to see their 30s, and all must endure a heavy burden of treatment and care that takes up hours of their lives every day, with regular hospital visits and stays.

We must now make a greater leap forward – and, today, we have a unique opportunity to accelerate change to bring forward the day we can say that everyone affected by CF can live a Life Unlimited by the condition.

One size doesn't fit all

This is an unprecedented moment for cystic fibrosis.

Today, the revolution in genomics, the increasing personalisation of healthcare and the unrelenting pace of digital innovation are driving dramatic advances in clinical research and empowering people to take greater control of their condition.

A pipeline of potentially transformational therapies driven by unparalleled levels of investment from the pharmaceutical industry offers new drugs focused on correcting the basic genetic cause of cystic fibrosis.

And new therapeutic techniques like genetic editing offer longer-term hope of an eventual cure.

The future is personal, and we must embrace this reality to accelerate innovations and realise the great opportunity that exists before us to make the giant leap we need towards a Life Unlimited.

Crucially, we also understand more about how CF affects each individual differently. Even those with the same genotype respond differently to targeted therapies – and there is a growing recognition of the influence of other physiological and environmental factors that determine its impact.

Add in the diverse needs and wishes of the expanding community of people with CF themselves, six out of 10 of whom are now aged 16 and over, many of whom are in work or study, and it is increasingly clear that a one-size fits-all approach to cystic fibrosis is no longer desirable, sufficient or sustainable.

New therapies, new care regimes, new interventions to support social and psychological wellbeing – as well as the range of information and advice to support people with CF at all stages of their lives – need to be tailored to the individual to have greatest impact.

Yet to do so we need a new approach and improved tools that not only harness the best clinical, scientific and commercial expertise but capture the knowledge, insight and experience of people with CF themselves.

If you are someone affected by CF today, your voice and your views have never been more important in driving change to improve and transform lives.

The future is personal, and we must embrace this reality to accelerate innovations and realise the great opportunity that exists before us to make the giant leap we need towards a Life Unlimited.

Our fight for a Life Unlimited: Our four flagship programmes

We are working on a range of fronts to support and empower people with CF and their families to enjoy better health and greater opportunities.

Our investment in world-class research programmes is focused on vital issues from pseudomonas infection to CF-related diabetes. We are supporting the clinical community to drive up quality of CF care in the NHS. We are providing vital information, advice and support to people affected by the condition throughout their lives. And we are campaigning for access to new therapies that can change lives.

But in order to seize the opportunity that exists to make a step-change in the fortunes of those with CF, we need to do more. We have therefore developed four flagship programmes to help drive long-term transformation.

They have been developed around three key tenets:

- **Empowering** people with CF (and those closest to them) to take control of their condition, using more robust data and providing crucial information and support where and when it's needed most.
- **Innovating** in all aspects of what we do across research, clinical care, information, support, lobbying and more..
- **Collaborating** with the entire community, from people with CF and their families to clinicians, researchers, parliamentarians and beyond, to provide the catalyst for the change we want to see.

Together, they are designed to help take a giant step forward in the development of a truly personalised approach.

Therapeutic Development Programme

Our Therapeutic Development Programme aims to help ensure people with CF get access to new therapies and innovations as quickly and as safely as possible. There is an unprecedented pipeline of potential therapies for people with CF being developed and more clinical trial capacity is needed to avoid slowing the progress of vital clinical research.

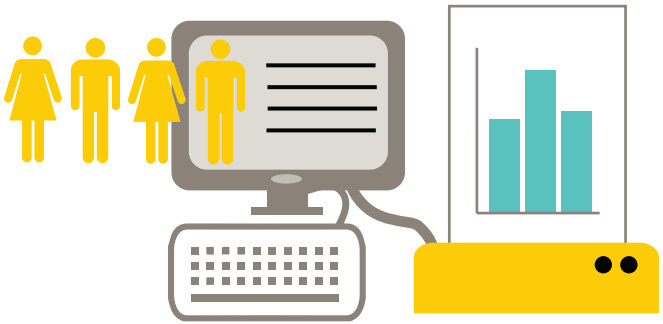
A key part of this programme is the creation of the Clinical Trials Accelerator Platform, a UK-wide initiative to bring together CF centres to increase participation and improve both the delivery of and equity of access to CF clinical trials in the UK, adding essential capacity across Europe and around the world.

Innovation Hubs

Innovation Hubs will bring together top talent from within existing research disciplines as well as those outside the biomedical arena like physics, maths, bioinformatics, nanotechnology and computer science to accelerate progress. These Hubs will act as a melting pot to bring this diverse range of extraordinary expertise to address and solve problems for CF, testing and exploring new ideas and approaches influenced by many different fields.

Alongside our existing research programme, we are exploring opportunities for high-level partnerships with world-class academic institutions to create a small number of CF-specific research institutes in the UK to build 'critical mass' capacity and expertise in order to lead national and international networks on key research issues. Receiving equivalent funds from the relevant academic institutions, they will also help to leverage significant additional funds into CF research from major funding institutions.

UK Cystic Fibrosis Registry



Our UK CF Registry securely contains detailed health information of more than 10,000 consenting individuals with CF in the UK. It is an extraordinary resource to help drive research and clinical improvement and the data contained within the Registry can be used to spot trends based on genotype, treatment, demographics and much more, as well as proving the real-world, long-term effect of treatments.

The Trust has undertaken a long-term investment in the development of the Registry and a new technical platform has been built to support its work to empower people with CF and enhance CF research. Clinical trials using the Registry will support new research, with plans being piloted to enable people with CF to securely access their own clinical data. By providing further personalised information, people with CF can help build a more complete picture of the realities of a life lived in a daily fight with the condition

SmartCareCF

SmartCareCF is a programme focussed on harnessing new technology to empower people with CF and develop potential new personalised solutions to improve care and research by the use of wearable devices and remote monitoring of health outcomes.

We are funding a multi-centre feasibility study of adults with CF using remote devices to monitor health outcome scores, and we are planning a parallel study aimed at young people too. The wider programme seeks to collaborate with industry, academics, the CF community and CF clinical centres to further innovate to help accelerate research, enhance self-management and reduce the treatment burden on people with cystic fibrosis.

More than the sum of their parts

Each one of these flagship programmes will, in themselves, help to drive change through empowerment, innovation and collaboration across the entire CF community. But their true strength lies in what they can achieve together.

Building on the foundation of the Trust's day-to-day work in research, clinical care, information and support, and campaigning, they will act as the catalyst and the driver for the fundamental shift we need to see as part of our fight for a Life Unlimited by cystic fibrosis.

Our goal is ambitious, but it is achievable. But we cannot achieve it alone. It will require the collective efforts of all parts of the CF community – from those with the condition themselves to clinical teams, from academics to industry. It is essential we seize this opportunity now.

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