Cystic Fibrosis Trust

Since 1964 | We won't stop until CF does

Accelerating CF Research

Strategies for rapidly progressing your science to clinical impact

Uniting for a life unlimited

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LifeArc

Accelerating CF Research

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Contents

Welcome

We're delighted to welcome you to our industry symposium. Cystic Fibrosis Trust funds and supports research to ensure that everyone with CF in the UK can live a long and full life. This aligns with the aims of LifeArc's Chronic Respiratory Challenge.

Throughout the morning you'll hear how colleagues from industry have received support from Cystic Fibrosis Trust to help drive their projects forward, which will include hearing more about our Clinical Trials Accelerator Platform and the UK CF Registry. During the networking lunch we hope that you'll take the opportunity to chat to us about how we can work with you to accelerate your research.

Dr Catherine Kettleborough
Head of Chronic Respiratory Infection
LifeArc

Dr Lucy Allen
Director of Research and Healthcare Data
Cystic Fibrosis Trust

Agenda

10.30–11am	**Registration, tea and coffee**
11–11.15am	**Welcome and introduction** Professor Jane Davies, Imperial College London Symposium Chair
11.15–11.20am	**Animation of Cystic Fibrosis Trust research support offer**
11.20–11.30am	**The power of CF community involvement** Case study from CF Antimicrobial Resistance Syndicate Dr Deborah O'Neil, NovaBiotics Ltd
11.30–11.40am	**Supporting research through partnerships and funding** Commercialising a novel medicine for CF through pre-clinical and clinical development with support from Cystic Fibrosis Trust Professor Janis Shute, Ockham Biotech Ltd
11.40–11.50am	**Supporting and delivering commercial clinical trials** Professor Alex Horsley, Manchester Adults CF Centre
11.50–12.00pm	**Driving research and informing CF care through healthcare data – working in partnership with UK CF Registry** Professor Nick Simmonds, Royal Brompton Hospital
12.00–12.30pm	**Panel Q&A** Dr Deborah O'Neil Professor Janis Shute Professor Alex Horsley Professor Nick Simmonds Dr Lucy Allen Dr Catherine Kettleborough
12.30–1.30pm	**Networking lunch**

Speaker biographies

Professor Jane Davies

Professor of Paediatric Respirology and Experimental Medicine and Honorary Consultant, National Heart and Lung Institute, Imperial College London

Jane has been global lead investigator on a large number of international trials of CFTR modulator drugs, including in children and infants. She is clinical lead and strategy group member in the UK CF Gene Therapy Consortium. She established and directs the Lung Clearance Core Facility on behalf of the European CF Society, standardizing this sensitive pulmonary outcome measure across multiple sites. She leads the UK NIHR CF National Research Strategy Group and is President of the European CF Society from the June conference.

Dr Deborah O'Neil

CEO, NovaBiotics, Aberdeen, UK

A biotechnology entrepreneur and immunologist by training, Deborah has 30 years of research experience; 20 of those in drug discovery and development. Deborah is CEO and founder of NovaBiotics, a business with a portfolio of first-in-class immunology-based therapy candidates for inflammatory, infectious and respiratory disease. Deborah chairs the UK's CF AMR Syndicate steering group, is a governing board member of IBioIC and was a board member of the UK's BIA and the BEAM Alliance. In 2020, Deborah was made OBE in the Queen's Birthday Honours list, for services to biotechnology, industry and charity.

Speaker biographies

Professor Janis Shute

Professor of Respiratory Pharmacology, University of Portsmouth

Janis is a Professor of Respiratory Pharmacology at the University of Portsmouth and Scientific Director of Ockham Biotech Ltd, a small commercial enterprise developing novel inhaled anti-inflammatory, mucolytic and antibiotic therapeutic approaches to clear the airways in people with cystic fibrosis.

Professor Alex Horsley

Professor of Respiratory Medicine, University of Manchester and Manchester Adult CF Centre

Alex is a Professor of Respiratory Medicine at the University of Manchester, Consultant at the Manchester Adult Cystic Fibrosis (CF) Centre, Director of the NIHR Manchester Clinical Research Facility, and until recently was Chair of Cystic Fibrosis Trust Clinical Trials Accelerator Platform (CTAP). Alex has led over 25 trials of new therapies in CF since 2012 and has been national or global lead on trials of several new therapies, including transformative CFTR modulator drugs. His academic work encompasses lung physiology and imaging (as clinical and trial outcomes), and lung infection.

Speaker biographies

Professor Nick Simmonds

Consultant Respiratory Physician and Associate Director of Royal Brompton Hospital Adult CF Centre, Royal Brompton Hospital/ Imperial College London

Nick is Associate Director of the Adult Cystic Fibrosis Centre at Royal Brompton Hospital, London, and Professor of Practice (Respiratory Medicine) at Imperial College London. His main research interests include difficult CF diagnosis and the investigation of novel CF therapies. He has been a lead investigator on numerous global multicentre trials and is Deputy Director of the European Cystic Fibrosis Society (ECFS) Clinical Trials Network. He has extensive experience of novel diagnostic techniques and is the Vice Coordinator of the ECFS Diagnostic Network. He is also Chair of the Registry Research Committee of the UK CF Registry, a role which promotes the use of registries to better understand outcomes in CF.

Dr Catherine (Katy) Kettleborough

Head of Chronic Respiratory Infection, LifeArc

Katy leads LifeArc's Chronic Respiratory Infection Translational Challenge. The Chronic Respiratory Infection Translational Challenge is an ambitious programme to accelerate scientific innovation for people living with bronchiectasis (BE) and cystic fibrosis (CF). This is done by working with partners, people with CF, academics, charities, healthcare professionals and industry to fast track scientific discoveries into new clinical solutions to transform how chronic respiratory infections are detected, treated and managed.

Speaker biographies

Dr Lucy Allen

Director of Research and Healthcare Data, Cystic Fibrosis Trust

Lucy joined Cystic Fibrosis Trust in late 2019. Lucy's previous experience includes building and leading research collaborations with industry, other research charities and clinical academics across a wide range of disease areas such a respiratory, mental health, and cancer and nutrition for the National Institute of Health Research (NIHR). She has also lead Research and Development projects for GE Healthcare developing radiopharmaceuticals for cancer and Alzheimer's disease. Lucy also has a PhD in lung inflammation and infection.

Cystic Fibrosis Trust research offer

Patient & Public Involvement and Engagement (PPIE)

Our involvement service can support collaborators to ensure appropriate and timely Patient & Public Involvement and Engagement activity is planned and facilitated, within the remit of any relevant codes of conduct, utilising the breadth of expertise that is held by our engaged network of CF PPIE contributors.

PPIE is well recognised for its impact on improving the quality of research, ensuring it focusses on what matters most to the community and enabling research to be designed in a feasible and accessible way.

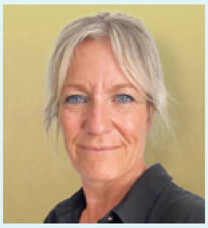
PPIE aims to occur at the earliest possible stages of the research pipeline, but can improve design, and therefore cost efficiency, at any time during the journey, particularly in relation to recruitment and retention rates in trials.

Its importance is recognised by UK regulatory bodies, who set expectations that sponsors undertake PPIE throughout the research process.

Talk to us, no matter where you are in your journey in CF research, we can identify, initiate, coordinate and facilitate the right activity for you!

Email: involvement@cysticfibrosis.org.uk
Website: Cystic Fibrosis Trust – community involvement

Key contacts



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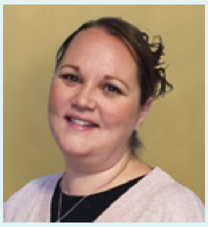

Claire Walter
PPIE Partnership Manager

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Cystic Fibrosis Trust research offer

Research funding

Cystic Fibrosis Trust supports and enables the delivery of a broad and dynamic portfolio of world-class innovative research that ensures that every person with CF in the UK can live a long and full life.

The Trust has several funding schemes including the Strategic Research Centre (SRC) and Development Award (DA) schemes, a summer studentship scheme as well as several co-funded fellowships schemes. In particular, our Venture and Innovation Awards provide financial support to innovative research projects.

Email: researchgrants@cysticfibrosis.org.uk
Website: Cystic Fibrosis Trust – apply for funding

Key contacts

Dr Paula Sommer
Head of Research Awards and Partnerships

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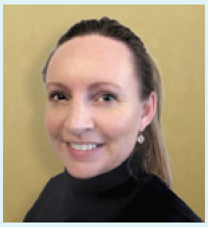

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Cystic Fibrosis Trust research offer

CF Antimicrobial Resistance (AMR) Syndicate partnership

CF AMR Syndicate was established in 2019. The Syndicate is jointly managed by Medicines Discovery Catapult, Cystic Fibrosis Trust and LifeArc. The CF AMR Syndicate is a cross-sector initiative that brings together leading experts in CF and AMR from industry, academia and the clinic with people living with CF.

The aim of the CF AMR Syndicate is to accelerate the development and translation of new CF antimicrobials and diagnostics to the clinic, bringing new medicines to people who need them.

Email: cfamr@md.catapult.org.uk
Website: CF AMR Syndicate

Key contacts

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Cystic Fibrosis Trust research offer

Clinical Trials Accelerator Programme (CTAP)

The Clinical Trials Accelerator Platform provides a robust clinical research infrastructure designed to support a diverse portfolio of CF studies with success and efficiency. It is part of a highly successful global trials network, including the European Clinical Trials Network and the Therapy Development Network in North America.

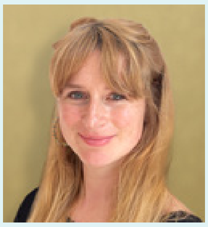
The infrastructure we have built, alongside the UK's proportionally large population of people with CF (~90% receive their care at a CTAP centre), creates a desirable location to open CF clinical trials.

We strive to support CF clinical research from bench to bedside and as such, a wide variety of phases and therapeutic areas – from early phase and genetic therapy through to real-world evidence.

CTAP's agile nature enables us to respond to the fast-changing, competitive clinical trials landscape. We take a flexible approach when it comes to supporting sponsors, tailoring support to the requirements of the study design, phase and therapeutic area. This ensures CF studies opening in the UK have the best chance of success.

Email: clinicaltrials@cysticfibrosis.org.uk
Website: Clinical Trials Accelerator Platform – information for sponsors

Key contacts



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Deborah Haworth
CTAP Business Development Manager

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Cystic Fibrosis Trust research offer

UK CF Registry

The UK CF Registry collects demographic, treatment and health outcome data on people with CF. Approximately 99% of people across the UK consent to sharing their data with the Registry.

The Registry can support feasibility and site selection for all phases of clinical trials, including early phase trials and is well connected to the Trust's Clinical Trials Accelerator Platform (CTAP).

Registry team expertise can support post-marketing pharmacovigilance studies (PASS and PAES) with long-term safety and efficacy monitoring. We can offer bespoke data collection options to tailor studies to your specific needs, ensuring regulatory compliance across the drug development journey.

The comprehensive dataset within UK CF Registry can be leveraged for pharmacoepidemiology studies, to explore drug utilisation patterns, identify potential drug interactions, and uncover real-world treatment effects.

The UK CF Registry data is recognised by the NICE Real-World Evidence Framework, strengthening your submissions with robust evidence on real-world effectiveness and cost-effectiveness for the NHS.

Email: registry@cysticfibrosis.org.uk
Website: Cystic Fibrosis Trust – UK CF Registry

Key contacts

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About LifeArc

LifeArc is a self-funded, not-for-profit medical research organisation and charity. We take science ideas out of the lab and help turn them into medical breakthroughs that can be life-changing for patients. We have been doing this for more than 25 years and our work has resulted in five licensed medicines, including cancer drug pembrolizumab (Keytruda®), lecanemab for Alzheimer's (Leqembi), and a diagnostic for antibiotic resistance.

Our teams are experts in drug and diagnostics discovery, technology transfer, and intellectual property. Our work is in translational science – bridging the gap between academic research and clinical development, providing funding, research and expert knowledge, all with a clear and unwavering commitment to having a positive impact on patient lives.

The LifeArc Chronic Respiratory Infection Translational Challenge (CRI TC) is our ambitious programme to accelerate scientific innovation for people living with bronchiectasis (BE) and cystic fibrosis (CF). By partnering with patients, academics, charities, healthcare professionals and industry, we aim to fast track scientific discoveries into new clinical solutions to transform how chronic respiratory infections are detected, treated and managed.

Find out more about our work on **www.lifearc.org**
or follow us on **LinkedIn** or **X (Twitter)**

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