

**Cystic
Fibrosis Trust**

UK CF Clinical Trials Accelerator Platform

A guide to successful CF clinical
research delivery in the UK

**Uniting for
a life unlimited**



Introduction

Launched in collaboration with the USA Cystic Fibrosis Foundation (CFF), the Clinical Trials Accelerator Platform (CTAP) is a UK-wide initiative bringing together paediatric and adult cystic fibrosis (CF) centres from around the UK to lead in the delivery of CF clinical trials. CTAP provides the infrastructure to support sponsors with delivery of CF clinical trials, and the platform to enable the CF community to gain access to these trials.

Through the set-up of CTAP there is now a well-established, collaborative and cohesive network of highly experienced and motivated CF clinical trial centres. Our network of 25 CTAP centres and national team of 25 CF trial coordinators have the expertise to support all types of CF trials, from early to late phase, from CFTR restoration to genetic therapies.



Through the CTAP network we support sponsors with:

- Patient-centred study design where 'Patient & Public Involvement' supports stronger study recruitment and retention
- Recruitment and referral centre identification utilising centre intelligence and demographic data
- Efficient study delivery through our network of 25 highly experienced CTAP centres, expert PI's and CF trial coordinators

Network of CTAP centres



25 centres form the CTAP network providing coverage of ~90% of the UK CF population. See Appendix on page 11 for details of the 25 CTAP centres.

The UK: a global partner in CF study delivery

Through working in partnership with the European Cystic Fibrosis Society's Clinical Trials Network (ECFS-CTN), and Cystic Fibrosis Foundation's Therapeutic Development Network (CFF-TDN), CTAP is part of a highly successful global trials network. In the UK, we have established a robust clinical research infrastructure designed to support and deliver a diverse portfolio of CF studies effectively and efficiently. The majority of CF care centres in the UK are either an active member of CTAP or have pathways in place to refer patients to CTAP centres for study participation.

CTAP also has the unique ability to work collaboratively with the **UK CF Registry** to support feasibility and identification of centres and trial participants across all UK CF care centres. This not only maximises recruitment potential, but also optimises equitability to CF studies for the majority of the UK CF community.



The infrastructure we have built, alongside the UK's proportionally large population of people with CF (more than 11,000, nearly 90% of which receive their care at a CTAP centre), creates a very desirable location to open high priority CF studies.

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

CTAP's agile nature enables the UK 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 as possible.

Our metrics illustrate the high level of engagement from both Industry and the CF community in UK-supported CF studies since the launch of CTAP in 2017:



- **Over 4,000** babies, children and adults have enrolled on to a CTAP study

- **Over 70** CF studies have been supported by CTAP so far

- Study set-up times have **decreased by 73%** across the CTAP network

- More than three quarters of studies have exceeded **90% of their recruitment target**

- The average **retention rate** for CTAP studies is **98%**



Overview of CTAP centre trial capabilities and experience

General

- Dedicated CF trial coordinators and experienced PIs
- Navigational support with UK regulatory systems, including devolved nations (England, Northern Ireland, Scotland, Wales)
- Excellent clinical research facilities including lung clearance index (LCI), MRI and CT scans
- Clinical trials pharmacy
- Sweat testing equipment and expertise
- Cardiopulmonary exercise testing
- Sputum induction and bronchoscopy expertise
- Digital technology and home monitoring capabilities



Specialist – early phase and genetic therapies

- National network of early phase capable CTAP centres with regional patient referral systems
- Dedicated CF early phase trial coordinators and PIs
- Experience of delivering early phase trials, including first-in-human
- Experience of advanced therapy medicinal products (ATMPs), including genetic therapies
- Capabilities to deliver inhaled genetic therapies
- Access to on-site nebulisation chambers for study drug dosing
- Facilities to support consecutive overnight stays with access to 24-hour nursing support

All CTAP centres have the opportunity to utilise the UK CF Registry to support identification of eligible study participants.

They also have established links with regional and national CF centres to facilitate patient referrals for clinical trial participation.



Sponsor support services

The infrastructure in the UK to support CF clinical research design, set-up and delivery is broad-ranging. CTAP not only sits within the centre of this rich clinical research ecosystem, but it plays a unique role in unifying the various services and streamlining support for sponsors.

Cystic Fibrosis Trust offers a **bespoke service to support sponsors** with all stages of CF study execution, from planning and design through to delivery and follow-up. Support services are **tailored to the needs of the CF study** and are designed to support as efficient and effective study delivery as possible.

Our support services link in highly specialised services from the **UK CF Registry** and **Community Involvement team** (network of CF patient representatives). This maximises the potential for CF studies opening in the UK, an increasingly valuable opportunity as the global and UK CF study landscape becomes increasingly competitive.



Engaging with our services early in the study planning process will have the biggest impact. Services offered may include the following:

Study design support

- Focus group and 1:1 interviews with members of the CF community whose lived experience will support **optimal study design** and participant-friendly study schedules
- Study documentation review by knowledgeable members of the CF community for patient-facing documents, helping to support **compliance with HRA regulatory requirements** for inclusion of patient & public involvement engagement (PPIE)
- Pre-feasibility data report from the UK CF Registry to support **identification and approximate location** of eligible patient populations
- **Navigational support** with UK regulatory systems
- Linkage with the National Institute for Health and Care Research (NIHR), **Respiratory Translational Research Collaboration** who support sponsors with early phase trial protocol development

Recruitment centre identification and selection

- Centralised, **data driven pre-feasibility** to identify the best placed centres to include in feasibility
- **Centralised and coordinated** feasibility assessment through CTAP and ECFS-CTN (for UK based centres)
- **Strategic and intelligence-based** centre selection support matching study requirements with patient populations and centres individual capabilities and capacity

Study delivery

- **25 experienced CTAP centres** with specialised team of PIs and CF trial coordinators to support **sponsors** with study set-up and delivery
- Collaborative network of CF centres with established **referral pathways** between UK CF centres (CTAP and non-CTAP)
- **Publicity of the study** via the **Trials Tracker** database to support the CF community to identify upcoming and current CF study opportunities

Post-study support

- Support with writing **lay summaries** for **study results**, results dissemination and **publicity** of published CF study results via the Trials Tracker
- Support with phase IV **post-marketing safety and efficacy studies** to support regulatory decision making and/or real-world evidence studies utilising the UK CF Registry

Summary of UK CF clinical research infrastructure

CF population

- Second largest CF population in the world
- CF representatives willing to share the voice of lived experience and expertise with sponsors to support CF study design and delivery through coordinated and facilitated Patient & Public Involvement & Engagement (PPIE) activity
- Research engaged CF community as demonstrated by the metrics on [page 5](#)

Expertise

- Internationally recognised PIs with expertise in commercial CF study delivery, including early phase and genetic therapies
- Highly experienced CF trial coordinators to lead in local study set-up, delivery and support with logistical issues

Infrastructure supporting study set-up and delivery

- Collaborative national CF clinical trials network
- Established referral pathways between CF centres
- UK CF Registry to support patient and recruitment centre identification

Promotional and engagement opportunities

- Opportunities to engage with CF clinical teams across the CTAP network through invitations to attend CTAP workshops and national CF clinical trial meetings
- Trials Tracker to publicise CF studies and study results
- Collaborative links with international networks CFF-TDN, ECFS-CTN and NIHR Respiratory Translational Research Collaboration

To discuss your CF study plans and explore how Cystic Fibrosis Trust can support your company, contact clinicaltrials@cysticfibrosis.org.uk.



Scan the above QR code for more info on our sponsor pages

Network of CTAP centres

England

Alder Hey Children's NHS Foundation Trust (Liverpool)	●	Royal Brompton and Harefield NHS Foundation Trust ¹ (London)	● ●
Barts Health NHS Trust (London)	● ●	Royal Devon University Healthcare NHS Foundation Trust	● ●
Blackpool Teaching Hospital NHS Foundation Trust	●	Royal Papworth Hospital NHS Foundation Trust ¹ (Cambridge)	●
Great Ormond Street Hospital for Children NHS Foundation Trust	●	Sheffield Children's NHS Foundation Trust	●
King's College Hospital NHS Foundation Trust ¹ (London)	● ●	South Tees Hospitals NHS Foundation Trust	●
Leeds Teaching Hospitals NHS Trust ¹	● ●	University Hospitals Birmingham NHS Foundation Trust ¹	● ●
Manchester University NHS Foundation Trust ¹	● ●	University Hospitals Bristol NHS Foundation Trust	● ●
Newcastle upon Tyne Hospitals NHS Foundation Trust	●	University Hospitals of Leicester NHS Trust	● ●
Norfolk and Norwich University Hospitals NHS Foundation Trust	●	University Hospitals of North Midlands NHS Trust	● ●
Nottingham University Hospitals NHS Trust ¹	● ●	University Hospital Southampton NHS Foundation Trust ¹	● ●
Oxford University Hospitals NHS Foundation Trust	●		

Northern Ireland

Belfast Health and Social Care Trust¹ ● ●

Scotland

NHS Greater Glasgow and Clyde¹ ● ●

NHS Lothian (Edinburgh) ● ●

Wales

Cardiff and Vale University Health Board¹ ● ●

Paediatric ● Adult ●



¹European Cystic Fibrosis Society Clinical Trial Network centre

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