Cystic Fibrosis Trust

UK CF Clinical Trials Accelerator Platform (CTAP)

Early phase trial programme

Uniting for a life unlimited

Clinical Trials Accelerator Platform

Launched in collaboration with the USA Cystic Fibrosis Foundation (CFF), the Clinical Trials Accelerator Platform (CTAP) is a UK-wide initiative bringing together cystic fibrosis (CF) centres from around the UK to support 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 timely access to these trials. It does this by:

- bringing together 27 CTAP centres experienced in running CF clinical trials
- providing ~90% of the UK CF population access to clinical trials – >4,000 babies and children and >5,000 adults
- funding a national team of 25 trial coordinators to support CF trial set-up and delivery
- creating a specialist network of CF early phase centres and trial coordinators.

CTAP's impact

Since the programme's launch in 2017:

Over 1,000

babies, children and adults with CF have enrolled on to a CTAP supported trial

42 CF trials

have been supported by the network of CTAP centres

26 CF trials

have been supported by CTAP with recruitment site identification and feasibility

Find out more by visiting
cysticfibrosis.org.uk/trialstracker

CTAP support for sponsors

CTAP provides sponsors with the following support for early and late phase clinical trials:

- national feasibility service based on specified demographics via the UK CF Registry, supporting country-level selection
- support with site identification based on the trial requirements
- centralised, centre-level feasibility service
- knowledge of setting up patient identification (referral) centres with established referral pathways
- facilitated Commercial Involvement (PPI) opportunities with our CF ambassadors, prior to and during study design supporting CF trials to become more participant-centred and less burdensome
- clinical Trials Digital Hub with CF Trials Tracker database listing all CF studies in set-up and open to recruitment.

See **cysticfibrosis.org.uk/trialstracker**

CTAP early phase programme

A specialist early phase CF network provides the infrastructure, resourcing and expertise to efficiently deliver phase 1 and 2a trials in the UK.

The early phase network includes six CTAP centres:

- Belfast Health and Social Care Trust (Northern Ireland)
- Cardiff and Vale Health Board (Wales)
- Manchester University NHS Foundation Trust (England)
- NHS Lothian (Scotland)
- Royal Brompton and Harefield NHS Trust (England)
- University Hospital Southampton NHS Trust (England)

CTAP early phase centres have:

- experience of delivering early phase trials, including FIH
- experience of ATMPs, including genetic therapies
- facilities to support consecutive overnight stays
- links with regional and national CF centres to facilitate patient referrals for early phase trial participation
- a network of experienced PIs who are experts in CF trial delivery, including early phase
- a dedicated early phase trial coordinator to oversee early phase trial set-up and delivery with access to 24-hour nursing support
- knowledge of UK regulatory set-up, including for each devolved nation
- infrastructure to support paediatric and adult early phase CF trials with specialist early phase trial facilities and dedicated clinic spaces
- formal agreement from the R&D department and National Institute for Health & Care Research (NIHR) Clinical Research Facilities (CRFs) to support early phase CF trials coming through the CTAP programme.

Why choose the UK to open early phase CF trials?

UK CF community demographics

~10,800 people with CF in the UK,

89% receive their care at a CTAP centre

47.7%
are homozygous F508d;

41.3%
are heterozygous F508d;

10.3%
have a rare variant*

60.6%
of people with CF in the UK are 16 years and older*

*Data taken from the UK CF Registry 2020 annual report

Overview of CTAP early phase trial capabilities (this list is not exhaustive)

- Lung clearance index (LCI)
- Lung MRI (structural & functional)
- PBMC collection and processing
- Bronchoscopy for research
- Ability to deliver nebulised gene therapies
- Ability to reconstitute gene therapies
- Pharmacy staff trained on cGMP

> **"The UK is a global player in clinical research, and we continue to punch above our weight in early phase trials."**
>
> The UK Department of Health and Social Care, November 2020

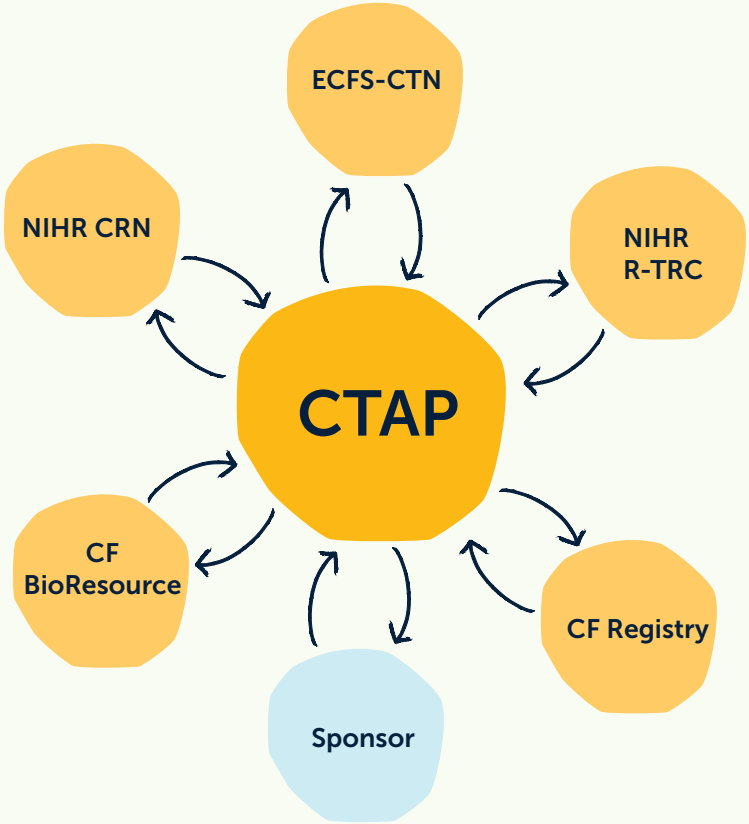
UK infrastructure to support sponsors with CF translational research delivery

The infrastructure in the UK to support CF clinical research 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 access for sponsors, helping navigation of the various pathways to support.

Two CF clinical trial networks operate collaboratively within the UK:

- CTAP and the European CF Society's Clinical Trials Network (ECFS-CTN)
- All 11 UK based ECFS-CTN centres are also part of CTAP, they are referred to as 'dual centres'



Both networks are aligned and operate collaboratively alongside these networks:

- NIHR BioResource – supports targeted identification of eligible, research-interested participants through collecting clinical and genetic data
- UK CF Registry – supports sponsors with design of post-marketing and pharmacovigilance studies, supports country-and site-level feasibility, hosts and embeds studies into the Registry to support data collection
- NIHR Respiratory Translational Research Collaboration (R-TRC) – provides sponsors with expert CF-specific advice and support for trial design and protocol development for translational research
- NIHR Clinical Research Network (CRN) – supports delivery of clinical trials through funding research staff and some study delivery costs

UK infrastructure overview

CTAP works closely with the NIHR and ECFS-CTN to ensure sponsors are signposted to the right support at the right time. The infrastructure in the UK ensures sponsors are well supported from the trial design stage onwards, helping ensure high quality new CF therapies are developed as efficiently as possible.

1. Protocol development

- **CTAP's Commercial Involvement** (PPI) platform provides opportunities for the CF community to support design of clinical trials from the conceptual stage onwards.
- **NIHR's Respiratory Translational Collaboration** (TRC) supports early stage protocol development.
- **ECFS-CTN** provides a protocol review service; a robust scientific review of trial design with the option for a global review with the Cystic Fibrosis Foundation (USA).

2. Centre identification & feasibility

In parallel, **CTAP** and the **ECFS-CTN** provide sponsors with support on identifying and selecting network centres who have the capacity and capabilities to meet the needs of the trial. The two networks also manage feasibility centrally.

3. Trial delivery

- **27 CTAP** centres and 25 trial coordinators support trial delivery, including six early phase trial centres and six early phase trial coordinators.
- 58 CF centres located in 17 countries across Europe form the **ECFS-CTN**, 11 of which are based in the UK (all of whom are also **CTAP** centres) and support trial delivery including early phase.
- **NIHR CF BioResource** supports targeted identification of eligible participants.
- **NIHR Clinical Research Facilities (CRFs)** provide resources (staff and facilities) to support early phase trials.

UK pathway for support with protocol development, review & adoption

Contact

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