

Longitudinal assessment of clinical measurements in patients with cystic fibrosis in preparation for a clinical trial of CFTR gene therapy

Submission date 12/05/2010	Recruitment status No longer recruiting	<input type="checkbox"/> Prospectively registered
Registration date 12/05/2010	Overall study status Completed	<input type="checkbox"/> Protocol
Last Edited 23/05/2017	Condition category Respiratory	<input type="checkbox"/> Statistical analysis plan
		<input type="checkbox"/> Results
		<input type="checkbox"/> Individual participant data
		<input type="checkbox"/> Record updated in last year

Plain English summary of protocol
Not provided at time of registration

Contact information

Type(s)
Scientific

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Additional identifiers

EudraCT/CTIS number

IRAS number

ClinicalTrials.gov number

Secondary identifying numbers
5028

Study information

Scientific Title

Longitudinal assessment of clinical measurements in patients with cystic fibrosis in preparation for a clinical trial of CFTR gene therapy

Acronym

Run-in study

Study objectives

The UK Cystic Fibrosis Gene Therapy Consortium (Imperial College, University of Edinburgh and Oxford) is working towards a clinical trial of CFTR gene therapy. The aim of this trial is to allow us to assess, for the first time, whether introducing normal copies of the CF gene can lead to clinical improvements. This aim of this trial imposes certain requirements on trial design:

1. The treatment will have to be given repeatedly over a period of time of sufficient length to allow clinical parameters to change
2. The clinical parameters used to assess response must be robust (repeatable, reproducible and sensitive to change)

The study registered here is the first part of this programme and does not involve administering any gene therapy agent to patients. It is to allow us to gain insight into the natural history of the assays we have designed for the future trial, to choose those assays and patients most likely to allow us to assess change sensitively and to provide pre-trial longitudinal data on the trial patients.

Ethics approval required

Old ethics approval format

Ethics approval(s)

ref: 07/Q0703/78

Study design

Multicentre non-randomised trial

Primary study design

Observational

Secondary study design

Multi-centre

Study setting(s)

Other

Study type(s)

Treatment

Participant information sheet

Health condition(s) or problem(s) studied

Topic: Respiratory, Generic Health Relevance and Cross Cutting Themes; Subtopic: Respiratory (all Subtopics), Generic Health Relevance (all Subtopics); Disease: Respiratory, Paediatrics

Interventions

200 CF patients aged 10 years and above will be recruited. All London patients will be recruited from the Royal Brompton Hospital adult and paediatric clinics. Adult patients in Scotland will be studied at the Western General Hospital, whilst the paediatric patients will be studied at the Royal Hospital for Sick Children. Scottish patients will be recruited from any Health Board in Scotland.

Over a period of approximately 2-3 years the patients will undergo five sets of measurements.

Study entry: registration only

Intervention Type

Other

Phase

Not Applicable

Primary outcome measure

To allow us to select assays for use in our future trial on the basis of their natural history

Secondary outcome measures

1. To provide informative data for future clinical trials
2. To provide clinical correlation

Overall study start date

17/09/2007

Completion date

01/07/2011

Eligibility

Key inclusion criteria

Not provided at time of registration

Participant type(s)

Patient

Age group

Not Specified

Sex

Not Specified

Target number of participants

Planned sample size: 200

Key exclusion criteria

Not provided at time of registration

Date of first enrolment

17/09/2007

Date of final enrolment

01/07/2011

Locations**Countries of recruitment**

England

United Kingdom

Study participating centre

Emmanuel Kaye Building

London

United Kingdom

SW3 6LR

Sponsor information**Organisation**

Imperial College London (UK)

Sponsor details

ICCH Building

59 North Wharf Road

London

England

United Kingdom

W2 1LA

Sponsor type

University/education

Website

<http://www3.imperial.ac.uk/>

ROR

<https://ror.org/041kmwe10>

Funder(s)

Funder type

Charity

Funder Name

Cystic Fibrosis Trust (UK)

Alternative Name(s)

Cystic Fibrosis, CF

Funding Body Type

Private sector organisation

Funding Body Subtype

Other non-profit organizations

Location

United Kingdom

Results and Publications

Publication and dissemination plan

Not provided at time of registration

Intention to publish date

Individual participant data (IPD) sharing plan

IPD sharing plan summary

Not provided at time of registration