

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	☐ Prospectively registered
Registration date 12/05/2010	Overall study status Completed	☐ Protocol
Last Edited 23/05/2017	Condition category Respiratory	☐ Statistical analysis plan
		☐ Results
		☐ Individual participant data
		☐ 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

Protocol serial number

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

Primary study design

Observational

Study design

Multicentre non-randomised trial

Study type(s)

Treatment

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(s)

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

Key secondary outcome(s)

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

Completion date

01/07/2011

Eligibility

Key inclusion criteria

Not provided at time of registration

Participant type(s)

Patient

Healthy volunteers allowed

No

Age group

Not Specified

Sex

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

United Kingdom

England

Study participating centre
Emmanuel Kaye Building
London
United Kingdom
SW3 6LR

Sponsor information

Organisation
Imperial College London (UK)

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

Funder(s)

Funder type
Charity

Funder Name
Cystic Fibrosis Trust (UK)

Alternative Name(s)
Cystic Fibrosis, cystic fibrosis (CF), CF

Funding Body Type
Private sector organisation

Funding Body Subtype
Trusts, charities, foundations (both public and private)

Location
United Kingdom

Results and Publications

Individual participant data (IPD) sharing plan

IPD sharing plan summary

