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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 [_] Protocol
Registration date 12/05/2010	**Overall study status** Completed	[_] Statistical analysis plan [_] Results
Last Edited 23/05/2017	**Condition category** Respiratory	[_] 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

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