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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

Registration date
12/05/2010

Overall study status
Completed

Last Edited
23/05/2017

Condition category
Respiratory

[_] Prospectively registered
[_] Protocol
[_] 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

Study design
Multicentre non-randomised trial

Primary study design
Observational

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