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Peripheral targeting of inhaled recombinant human deoxyribonuclease in stable cystic fibrosis patients

Submission date	Recruitment status	[X] Prospectively registered
07/03/2007	No longer recruiting	[ ] Protocol
Registration date	**Overall study status**	[ ] Statistical analysis plan
07/03/2007	Completed	[ ] Results
Last Edited	**Condition category**	[ ] Individual participant data
12/08/2008	Nutritional, Metabolic, Endocrine	[ ] 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
2412325-3; NTR912

Study information

Scientific Title

Study objectives
Recombinant human deoxyribonuclease (rhDNase) targeted to the peripheral airways improves lung function in children with cystic fibrosis (CF) and a stable clinical condition.

Ethics approval required
Old ethics approval format

Ethics approval(s)
Ethics approval received from the Medical Ethical Committee of Erasmus MC Rotterdam on the 26th April 2007.

Study design
Randomised, active controlled, parallel group, double blinded, multicentre trial

Primary study design
Interventional

Secondary study design
Randomised controlled trial

Study setting(s)
Hospital

Study type(s)
Treatment

Participant information sheet

Health condition(s) or problem(s) studied
Cystic fibrosis

Interventions
25 patients will receive four weeks of treatment with inhaled rhDNase targeted to the peripheral airways and 25 patients will receive four weeks of treatment with inhaled rhDNase targeted to the central airways. The central airways regimen is aimed to simulate equal deposition pattern as compared to conventional maintenance therapy. The peripheral airway regimen deposits a greater percentage of the medication in the peripheral airways.

Intervention Type
Drug

Phase

Not Specified

Drug/device/biological/vaccine name(s)
Recombinant human deoxyribonuclease (rhDNase)

Primary outcome measure
Primary endpoint will be the change in forced expiratory flow (FEF75) as a result of treatment. FEF75 is the most suitable endpoint since it is sensitive to peripheral airways obstruction.

Secondary outcome measures
Secondary endpoints will include:
1. Lung Clearance Index (LCI) measurements as assessed by multiple breath washout
2. Other values obtained in the flow volume curve:
2.1. Maximum mean expiratory flow (MMEF25-75)
2.2. Forced expiratory volume in one second (FEV1)
2.3. Forced Vital Capacity (FVC)
3. Other study parameters, such as use of antibiotics and number of exacerbations (if applicable)

Overall study start date
01/05/2007

Completion date
01/05/2008

Eligibility

Key inclusion criteria
1. Age between six and 18 years old
2. Diagnosis of CF confirmed by sweat-test and/or deoxyribonucleic acid (DNA) analysis and/or electro-physiology testing (nasal potential difference measurement)
3. Routine treatment with rhDNase once daily, started at least one month before enrolment in the study
4. Stable condition, in this study defined as: no intravenous (i.v.) antibiotics (hospital or at home) in the previous month and constant medication regime during the previous two weeks (for example: no additional oral antibiotics course, no newly started inhaled or systemic corticosteroids etc.,)
5. Ability to perform lung function tests (assessed by trained lung function technician)
6. Lung function: forced vital capacity (FVC) greater than 40% predicted
7. Signed written informed consent

Participant type(s)
Patient

Age group
Child

Lower age limit
6 Years

Upper age limit

18 Years

Sex
Not Specified

Target number of participants
50

Key exclusion criteria
1. Inability to follow instructions of the investigator
2. Inability to inhale rhDNase
3. Clinical condition not stable, as assessed by the patients paediatrician
4. Concomitant medical conditions that effect inhaled treatment (e.g. cleft palate, severe malacia)
5. Current respiratory tract infection
6. Pulmonary complications that might put the patient at risk to participate in the study
7. Neuromuscular disease
8. Poor compliance with treatment as assessed by the patients paediatrician
9. Active allergic bronchopulmonary aspergillosis (ABPA) defined as an oral course of prednisone for ABPA within the last three months

Date of first enrolment
01/05/2007

Date of final enrolment
01/05/2008

Locations

Countries of recruitment
Italy

Netherlands

Study participating centre
Division of Pediatric Respiratory Medicine, Room Sb-2666
Rotterdam
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3015 GJ

Sponsor information

Organisation
Erasmus Medical Centre (The Netherlands)

Sponsor details
Sophia Children's Hospital
Dr. Molewaterplein 60
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Netherlands
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Sponsor type
Hospital/treatment centre

Website
http://www.erasmusmc.nl/

ROR
https://ror.org/018906e22

Funder(s)

Funder type
Industry

Funder Name
Roche Nederland B.V. (The Netherlands)

Funder Name
Erasmus Medical Centre (The Netherlands)

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