

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

Submission date 07/03/2007	Recruitment status No longer recruiting	☒ Prospectively registered
Registration date 07/03/2007	Overall study status Completed	☐ Protocol
Last Edited 12/08/2008	Condition category Nutritional, Metabolic, Endocrine	☐ 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
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.

Primary study design

Interventional

Study design

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

Study type(s)

Treatment

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(s)

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.

Key secondary outcome(s)

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)

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

Healthy volunteers allowed

No

Age group

Child

Lower age limit

6 Years

Upper age limit

18 Years

Sex

Not Specified

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

Netherlands

3015 GJ

Sponsor information

Organisation

Erasmus Medical Centre (The Netherlands)

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

Individual participant data (IPD) sharing plan

IPD sharing plan summary

Not provided at time of registration