Hypertonic saline in primary ciliary dyskinesia: a pilot study

Published: 25-07-2013
Last updated: 23-04-2024

We hypothesize that hypertonic saline nebulizations improve respiratory symptoms and quality of life in PCD patients.

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Respiratory disorders congenital
Study type	Interventional

Summary

ID

NL-OMON40843

Source
ToetsingOnline

Brief title
Hypertonic saline in primary ciliary dyskinesia

Condition

- Respiratory disorders congenital
- Bacterial infectious disorders
- Respiratory tract infections

Synonym
Kartagener syndrome, Primary ciliary dyskinesia

Research involving
Human

Sponsors and support

Primary sponsor: Vrije Universiteit Medisch Centrum
Source(s) of monetary or material Support: PCD patiëntenorganisatie

Intervention

Keyword: Bronchiectasis, Hypertonic saline, Primary ciliary dyskinesia, Treatment

Outcome measures

Primary outcome

Change in St. George's Respiratory Questionnaire score.

Secondary outcome

Change in Quality of Life questionnaire for bronchiectasis patients score, lung function, lung clearance index, sputum culture results, inflammatory markers in sputum and blood, exacerbation frequency.

Study description

Background summary

Primary ciliary dyskinesia (PCD) is an autosomal recessive disorder, characterized by dysfunction of the cilia. Cilia line the respiratory epithelium and form the basis of effective mucociliary clearance. Patients experience recurrent respiratory tract infections leading to permanent lung damage. At present, there are no randomized controlled trials on the treatment of patients with primary ciliary dyskinesia. Treatment is largely based on expert opinions and is focused on the control of pulmonary exacerbations. Protocols are derived from cystic fibrosis (CF) guidelines. However, CF has a different pathophysiology and therefore responses to treatments may be different. Hypertonic saline has been shown to improve mucociliary clearance in CF patients, but also in asthma and healthy controls. In CF patients bi-daily nebulizations wit hypertonic saline improves lung function and reduces respiratory symptoms and exacerbations. However, studies in non-CF bronchiectasis patients are limited and the results are contradicting

Study objective

We hypothesize that hypertonic saline nebulizations improve respiratory symptoms and quality of life in PCD patients.

Study design

This is a randomized placebo-controlled double-blind crossover pilot study, conducted at the VU University Medical Center (VUmc), Amsterdam and the Academic Medical Center (AMC), Amsterdam. The study has an AB/BA crossover design with two interventions.

Intervention

A Hypertonic saline (7%) inhalation bi-daily during 3 months
B Placebo: Isotonic saline (0.9%) inhalation bi-daily during 3 months

Study burden and risks

Safety and efficacy of hypertonic saline nebulizations have been observed in children and adults with CF and non-CF bronchiectasis. This study consists of 6 study visits of which half can be combined with standard 3-monthly outpatient clinic visits. During these visits patients will have their medical history taken and will undergo a physical examination, lung function tests, sputum induction and blood withdrawal. There are only minor risks associated with this study. Patients could directly benefit from the effect of the hypertonic saline during the study.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

o Diagnosis of primary ciliary dyskinesia
o * 18 years of age
o Capable of performing lung function tests.
o The forced expiratory volume in one second (FEV1), measured at screening, has to be within 10 % of the best value obtained during the previous six months and at least 40% of the predicted value for height, age and sex.

Exclusion criteria

o Smoking
o FEV1 < 40 %.
o Use of Pulmozyme or other mucolytics or non-routine antibiotics in the previous 30 days.
o A decline in lung function of more than 15 % or oxyhemoglobin of < 90% after test nebulisation with hypertonic saline at screening visit17.
o Women with a current or intended pregnancy during the trial
o Diagnosis of quinine sulphate allergy
o Myasthenia Gravis
o Lambert-Eaton syndrome
o Optic neuritis
o Tinnitus
o Atrium fibrillation and other currently severe cardiac disease
o Epilepsy
o Glucose 6PD deficiency

Study design

Design

Study phase:	4
Study type:	Interventional
Intervention model:	Crossover

Allocation:	Randomized controlled trial
Masking:	Double blinded (masking used)
Control:	Placebo
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	15-09-2013
Enrollment:	20
Type:	Actual

Ethics review

Approved WMO Date:	25-07-2013
Application type:	First submission
Review commission:	METC Amsterdam UMC
Approved WMO Date:	23-08-2013
Application type:	First submission
Review commission:	METC Amsterdam UMC
Approved WMO Date:	11-08-2014
Application type:	Amendment
Review commission:	METC Amsterdam UMC

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
EudraCT	EUCTR2013-001020-19-NL
CCMO	NL42765.029.13
Other	NTR candidate nr 14470