A trial to determine the efficacy of dry powder mannitol in improving lung function in subjects with Cystic Fibrosis aged six to seventeen years

No registrations found.

Ethical review	Positive opinion
Status	Recruiting
Health condition type	-
Study type	Interventional

Summary

ID

NL-OMON21839

Source
NTR

Brief title
DPM-CF-204

Health condition

cystic fibrosis, mucoviscidosis, taaislijm ziekte

Sponsors and support

Primary sponsor: Pharmaxis Ltd
Source(s) of monetary or material Support: Pharmaxis Ltd

Intervention

Outcome measures

Primary outcome

The absolute change from treatment periodbaseline to week 8 of each treatment period in

percentage of predicted FEV1.

Secondary outcome

Change from treatment period baseline in percentage of predicted FVC t
Change from treatment period baseline in percentage of predicted FEF25-75 (exploratory endpoint)
Adverse events, vital signs and physical examination
Treatment induced sputum weight

Study description

Study objective

It is hypothesised that inhaled mannitol 400 mg b.d. will lead to a significant improvement in the absolute change in percentage of predicted FEV1 from baseline following eight-weeks of trial treatment compared to treatment with inhaled placebo b.d.
Any improvement in FEV1 is considered clinically meaningful; however, this trial has set a threshold of 3% for the purposes of determining an appropriate sample size for statistical power whilst retaining trial feasibility in an orphan disease population.

Study design

Trial started 21 Jun 2013
Trial end planned 11 Mar 2015

Intervention

Study Drug = Mannitol 400mg/twice a day
Placebo = non active Mannitol 400mg/twice a day, that means: the placebo consists of larger particles of Mannitol and therefore it is not inhaled into the lungs

The study drug is administered via a dry powder inhaler.
• Mannitol 400 mg twice a day for 8 weeks, followed by an 8-week washout period followed by 400 mg placebo /twice a day for 8 weeks
or
• 400 mg placebo twice a day for 8 weeks, followed by an 8-week washout period, followed by Mannitol 400 mg/twice a day for 8 weeks.

Contacts

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Eligibility criteria

Inclusion criteria

1. Personally provide, or have a legal guardian provide written informed consent to participate in the trial, according to local regulations;
2. rhDNase and maintenance antibiotic use is allowed but treatment must have been established at least 3 months prior to screening. The subject must remain on rhDNase and / or maintenance antibiotics for the duration of the trial. The subject must not commence treatment with rhDNase or maintenance antibiotics during the trial;
3. Have a confirmed diagnosis of cystic fibrosis (sweat test result ¡Ý 60 mEq/L chloride and/or genotyping showing two identifiable mutations consistent with a diagnosis of cystic fibrosis);
4. Be aged ¡Ý 6 years and < 18 years;
5. Have a percentage of predicted FEV1 of ¡Ý 30% and ¡Ü 90% at Screening (Visit 0). Percentage of predicted FEV1 will be calculated using Wang for children aged < 8 years, and using NHanes III for those ¡Ý 8 years; and
6. Be able to perform all the techniques necessary to measure lung function.

Exclusion criteria

1. Be using maintenance nebulised hypertonic saline;
2. Be considered ¡°terminally ill¡±; eligible for lung transplantation, or have received a lung transplant previously;
3. Require home oxygen or assisted ventilation;
4. Have had an episode of massive haemoptysis defined as acute bleeding ¡Ý 240 ml in a 24-hour period and/or recurrent bleeding ¡Ý100 ml/day over several days in the three-months prior to Screening (Visit 0);

5. Have a known intolerance to mannitol;
6. Be taking non-selective ¦Â blockers;
7. In the three months prior to Screening (Visit 0) have had a myocardial infarction; a cerebral vascular accident; major ocular, abdominal, chest or brain surgery;
8. Have a known cerebral, aortic or abdominal aneurysm;
9. Be currently participating in, or have participated in another investigative drug trial within four weeks of Screening (Visit 0);
10. Be pregnant or breastfeeding, or plan to become pregnant whilst in the trial;
11. For females of childbearing potential, be using an unreliable form of contraception (at the discretion of the investigator);
12. Have any concomitant medical, psychiatric, or social condition that, in the Investigator¡¯s opinion, would put the subject at significant risk, may confound the results or may significantly interfere with the subject¡¯s participation in the trial; or
13. Have a ¡°failed¡± or ¡°incomplete¡± mannitol tolerance test.

Study design

Design

Study type:	Interventional
Intervention model:	Crossover
Allocation:	Randomized controlled trial
Masking:	Double blinded (masking used)
Control:	Placebo

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	21-06-2013
Enrollment:	160
Type:	Anticipated

Ethics review

Positive opinion

Date:	27-02-2014
Application type:	First submission

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
NTR-new	NL4215
NTR-old	NTR4453
Other	NCT01883531 : DPM-CF-204

Study results