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

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Ethische beoordeling	Positief advies
Status	Werving gestart
Type aandoening	-
Onderzoekstype	Interventie onderzoek

Samenvatting

ID

NL-OMON21839

Bron
Nationaal Trial Register

Verkorte titel
DPM-CF-204

Aandoening

cystic fibrosis, mucoviscidosis, taaislijm ziekte

Ondersteuning

Primaire sponsor: Pharmaxis Ltd
Overige ondersteuning: Pharmaxis Ltd

Onderzoeksproduct en/of interventie

Uitkomstmaten

Primaire uitkomstmaten

The absolute change from treatment periodbaseline to week 8 of each treatment period in percentage of predicted FEV1.

Toelichting onderzoek

Doel van het onderzoek

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.

Onderzoeksopzet

Trial started 21 Jun 2013
Trial end planned 11 Mar 2015

Onderzoeksproduct en/of interventie

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.

Contactpersonen

Publiek

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Wetenschappelijk

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Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

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.

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

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.

Onderzoeksopzet

Opzet

Type:	Interventie onderzoek
Onderzoeksmodel:	Cross-over
Toewijzing:	Gerandomiseerd
Blindering:	Dubbelblind
Controle:	Placebo

Deelname

Nederland

Status:	Werving gestart
(Verwachte) startdatum:	21-06-2013
Aantal proefpersonen:	160
Type:	Verwachte startdatum

Ethische beoordeling

Positief advies

Datum:	27-02-2014
Soort:	Eerste indiening

Registraties

Opgevolgd door onderstaande (mogelijk meer actuele) registratie

Geen registraties gevonden.

Andere (mogelijk minder actuele) registraties in dit register

Geen registraties gevonden.

In overige registers

Register	ID
NTR-new	NL4215
NTR-old	NTR4453
Ander register	NCT01883531 : DPM-CF-204

Resultaten