STEPS study

Gepubliceerd: 19-12-2013 Laatst bijgewerkt: 15-05-2024

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Ethische beoordeling	Positief advies
Status	Werving gestart
Type aandoening	-
Onderzoekstype	Observationeel onderzoek, zonder invasieve metingen

Samenvatting

ID

NL-OMON20636

Bron
Nationaal Trial Register

Verkorte titel
STEPS study

Aandoening

Sarcoidosis, sarcoidose, prednisone, prednison, treatment, behandeling, pulmonary function, longfunctie

Ondersteuning

Primaire sponsor: ErasmusMC
Overige ondersteuning: Sponsor

Onderzoeksproduct en/of interventie

Uitkomstmaten

Primaire uitkomstmaten

1. Repeated measurements of the FVC and prednisone dose used

Toelichting onderzoek

Achtergrond van het onderzoek

Sarcoidosis is a granulomatous disorder of unknown cause. Current therapy is immunosuppressive, not curative and often ineffective, as we do not thoroughly understand the underlying pathogenesis. Oral corticosteroids are the first line of therapy in pulmonary sarcoidosis patients with significant pulmonary symptoms and/or progressive disease as determined by radiology or lung function. In the latter case, treatment is primarily aimed at preventing organ damage, although previous studies did not conclusively demonstrate a beneficial effect in preventing disease progression or pulmonary fibrosis. Nevertheless, current treatment guidelines advocate relatively long and high initial treatment regimes, without clear evidence for the optimal dose and duration of treatment. Importantly, prolonged treatment with prednisone is known to induce considerable side-effects/co-morbidity.
Several expert-opinions suggest a lower initial dose and shorter initial phase, based on retrospective case series. However, there is considerable variety in the response to treatment between sarcoidosis individuals and immunological mechanisms underlying this phenomenon have not been elucidated yet. These data suggest that treatment of sarcoidosis patients should be individualized, pursuing the lowest prednisone dose for the shortest period possible. However, prospective data on the early response towards prednisone treatment and tapering is lacking in a well-characterized cohort of pulmonary sarcoidosis patients.
Against this background, we aim to describe the pulmonary response to corticosteroid therapy and tapering in a cohort of newly treated pulmonary sarcoidosis patients, using a hand-held spirometer. With results of this study, in the future, we hope to individualize the treatment and tapering strategy for sarcoidosis patients. This could lead to prednisone dose sparing, reduction in co-morbidity and an increased quality of life of sarcoidosis patients. This treatment strategy has already been performed successfully in other chronic pulmonary diseases requiring prednisone treatment, like asthma.

Doel van het onderzoek

Sarcoidosis is a granulomatous disorder of unknown cause. Current therapy is immunosuppressive, not curative and often ineffective, as we do not thoroughly understand the underlying pathogenesis. Oral corticosteroids are the first line of therapy in pulmonary sarcoidosis patients with significant pulmonary symptoms and/or progressive disease as determined by radiology or lung function. In the latter case, treatment is primarily aimed at preventing organ damage, although previous studies did not conclusively demonstrate a beneficial effect in preventing disease progression or pulmonary fibrosis. Nevertheless, current treatment guidelines advocate relatively long and high initial treatment regimes, without clear evidence for the optimal dose and duration of treatment. Importantly, prolonged treatment with prednisone is known to induce considerable side-effects/co-morbidity.

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Against this background, we aim to describe the pulmonary response to corticosteroid therapy and tapering in a cohort of newly treated pulmonary sarcoidosis patients, using a hand-held spirometer. With results of this study, in the future, we hope to individualize the treatment and tapering strategy for sarcoidosis patients. This could lead to prednisone dose sparing, reduction in co-morbidity and an increased quality of life of sarcoidosis patients. This treatment strategy has already been performed successfully in other chronic pulmonary diseases requiring prednisone treatment, like asthma.

Onderzoeksopzet

Baseline visit, months 1,3,6,9,12

Onderzoeksproduct en/of interventie

Patients participating in this study will be treated according to current guidelines. As such, diagnostic procedures or treatment will not be postponed during participation. Standard procedures will be executed during regular visits at the outpatient clinic. In course of the research:

1. Daily home monitoring of the pulmonary function (PF) will be performed with a hand-held spirometer (MicroDiary, CareFusion) during the first three months of treatment;

2. The patient is asked to keep record of his/her symptomatic response to therapy by filling out a weekly dyspnoea score (MRC scale) and a fatigue score (FAS score) during the first three months of treatment at home;

3. During the regular visits at the outpatient clinic, patient reported response to therapy will be objectified by two standardized questionnaires (SGRQ, SF-36, KSQ), in-hospital pulmonary function tests at the department of pulmonology, a chest X-ray, thorax-HRCT (only if physician’s choice), weight and laboratory data will be recorded;

4. The patient will be asked to donate an additional 60 ml peripheral blood for immunological

analysis at the baseline visit, month 1,3 and 12 (i.e. amount of macrophages, NK-cells, dendritic cells, T-cells, B-cells and cytokines; apoptotic susceptibility towards FasL, IL-2 deprivation and prednisone; expression of activation, differentiation and apoptotic markers; immunosuppressive function of lymphocyte subsets and HLA-type will be examined). Blood will be collected during routine venapunction.

Contactpersonen

Publiek

Department of Pulmonary Medicine,
dr. Molenwaterplein 50
Caroline Broos
Rotterdam 3015 GE
The Netherlands
010-7043701

Wetenschappelijk

Department of Pulmonary Medicine,
dr. Molenwaterplein 50
Caroline Broos
Rotterdam 3015 GE
The Netherlands
010-7043701

Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

-Patients with stage II/III sarcoidosis (based on a chest X-ray or equivalent on a CT-scan) (established using the criteria of the World Association of Sarcoidosis and Other Granulomatous Disorders (WASOG));

- A forced vital capacity (FVC) < 80% of predicted AND/OR an absolute decline of > 10% predicted PVC within 12 months

- A pulmonary indication for prednisolone treatment (determined by the treating physician

and conform current clinical guidelines);

- Written informed consent;

- Age > 18.

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

- Previous immunosuppressive treatment for sarcoidosis (e.g. prednisone, methotrexate (MTX), Infliximab);

- Use of systemic immunosuppressive therapy within the previous 3 months for another disease then sarcoidosis;

- Contra-indication for steroids;

- Other conditions which could influence pulmonary function, such as:
o Concurrent chronico bstructive pulmonary disorder(COPD);

o Concurrent active asthma (according to GINA guidelines);

o Fibrotic lung disease;

o Pregnancy;

o Morbideobesitas(BMI>30);

- Pulmonary malignancies.

Onderzoeksopzet

Opzet

Type:	Observationeel onderzoek, zonder invasieve metingen
Onderzoeksmodel:	Factorieel
Toewijzing:	Niet-gerandomiseerd

Controle: N.v.t. / onbekend

Deelname

Nederland

Status:	Werving gestart
(Verwachte) startdatum:	19-12-2013
Aantal proefpersonen:	50
Type:	Verwachte startdatum

Voornemen beschikbaar stellen Individuele Patiënten Data (IPD)

Wordt de data na het onderzoek gedeeld: Nog niet bepaald

Ethische beoordeling

Positief advies

Datum:	19-12-2013
Soort:	Eerste indiening

Registraties

Opgevolgd door onderstaande (mogelijk meer actuele) registratie

ID: 44859
Bron: ToetsingOnline
Titel:

Andere (mogelijk minder actuele) registraties in dit register

Geen registraties gevonden.

In overige registers

Register	ID
NTR-new	NL4169
NTR-old	NTR4328
CCMO	NL44805.078.13
ISRCTN	ISRCTN wordt niet meer aangevraagd.
OMON	NL-OMON44859

Resultaten

Samenvatting resultaten
N/a