Defining treatment response in patient with CIDP, shifting from statistics to bedside

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Ethical review	Approved WMO
Status	Recruiting
Health condition type	Peripheral neuropathies
Study type	Observational non invasive

Summary

ID

NL-OMON48408

Source
ToetsingOnline

Brief title
MCID in CIDP

Condition

- Peripheral neuropathies

Synonym
Chronic Inflammatory Demyelinating Polyneuropathy, CIDP

Research involving
Human

Sponsors and support

Primary sponsor: Amsterdam UMC, locatie AMC
Source(s) of monetary or material Support: Prinses Beatrix Spierfonds

Intervention

Keyword: Chronic Inflammatory Demyelinating Polyneuropathy, CIDP, minimal clinically important difference (MCID), treatment

Outcome measures

Primary outcome

To define treatment response in CIDP using patient pMCID versus traditional

MCID cut-offs on a linear disability scale.

Secondary outcome

1) To determine whether pMCID during improvement is larger than the pMCID

during deterioration.

2) To determine the margins of score fluctuation on the I-RODS during follow-up

in patients with stable disease.

Study description

Background summary

Currently, improvement and deterioration in chronic inflammatory demyelinating polyradiculoneuropathy (CIDP) is defined by clinical outcome measures because biomarkers of disease activity are lacking. Despite various outcome measures, it remains unclear when patients should be considered a *responder to treatment*. Better definitions of *responder to treatment* are needed to design better trials and identify new treatment strategies, but also to provide guidance to tailor treatment in daily practice. Ideally, patients* voice should be centralized in the definition of treatment response, rather than relying on fixed and predefined cut-offs currently (statistical MCID or sMCID) used in clinical trial designs.

Study objective

Primary objective is to define and validate a patient perceived minimal clinical important difference (pMCID) on a linear disability scale. Secondary objectives are to determine whether pMCID during improvement is larger compared to the pMCID during deterioration and to determine the margins of natural

fluctuation on disability scales during follow-up of patients with stable disease.

Study design

Observational longitudinal study with a follow-up of 24 weeks

Study burden and risks

The burden associated with this observational study is limited to four to six self -assessed measurements at home consisting of completing questionnaires and measuring grip strength plus three to four visits to the hospital, which will be embedded as part of the routine evaluation of patients otherwise performed outside the study. Part of the outcome measures used in this study are also used in routine clinical practice. There are no risks related to participation in this 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

Possible, probable or definite clinical and electrophysiological EFNS/PNS criteria for CIDP (Group 1 and 2).5 In addition:
A) Patients in Group 1 need to have active disease (history of progression justifying start or change of treatment as determined by treating physician).
B) Patients in Group 2 with stable disease for three months on treatment with IVIg and/or corticosteroids.
OR
have a polyneuropathy associated with monoclonal IgM paraproteinemia (with or without anti-MAG antibodies) (Group 3, controls).
- Adult males or females (18 year or more).

Exclusion criteria

- Any concomitant disease (e.g. joint problems) or condition (e.g. misuse of alcohol) that might interfere with functionality of the patient, which could potentially interfere with capturing MCID changes related predominantly to CIDP.
- Legally incompetent adults
- No informed consent

Study design

Design

Study type:	Observational non invasive
Intervention model:	Other
Allocation:	Non-randomized controlled trial
Masking:	Open (masking not used)

Primary purpose: Other

Recruitment

NL
Recruitment status: Recruiting

Start date (anticipated):	03-09-2019
Enrollment:	120
Type:	Actual

Ethics review

Approved WMO Date:	04-06-2019
Application type:	First submission
Review commission:	METC Amsterdam UMC
Approved WMO Date:	12-12-2019
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
CCMO	NL69897.018.19