EXPLORE-SFN Diagnostics: EXtensive Patient LOngitudinal data REgistry for Small Fiber Neuropathy Diagnostics

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The primary objective is to gain information regarding the natural course of SFN and the change over time of the diagnostics using clinimetrically sound outcome measures representing various levels of outcome assessment. The secondary objective is...

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

Summary

ID

NL-OMON53455

Source
ToetsingOnline

Brief title
EXPLORE-SFN Diagnostics

Condition

- Peripheral neuropathies

Synonym
Small fiber neuropathy

Research involving
Human

Sponsors and support

Primary sponsor: Medisch Universitair Ziekenhuis Maastricht
Source(s) of monetary or material Support: Prinses Beatrix Spierfonds

Intervention

Keyword: Natural course, Outcome measures, Registry, Small fiber neuropathy

Outcome measures

Primary outcome

The primary endpoint is an electronic web-based registry, which reflects the natural course of SFN and change over time in the diagnostics of SFN. Changes in neurological examination, nerve conduction studies, IENFD, quantitative sensory testing and their relation to SFN-related symptoms, daily functioning and QoL will be demonstrated.

Secondary outcome

The secondary endpoint is if and what diagnostics could ultimately be used in future trials.

Study description

Background summary

Small fiber neuropathy (SFN) is characterized by neuropathic pain and dysautonomia and may lead to limitations in activity and participation with a decrement in quality of life (QoL). Increasing knowledge in the underlying pathophysiological mechanisms leads to the development of more targeted therapies. However, before treatments can be investigated in clinical trials, a better understanding regarding the natural clinical course is needed. In addition, it is crucial to determine whether applied outcome measures show sensitivity to capture what is considered clinically meaningful responsiveness. A national registry for SFN patients including diagnostics will provide long-term follow-up data to achieve clinical trial readiness.

Study objective

The primary objective is to gain information regarding the natural course of SFN and the change over time of the diagnostics using clinimetrically sound outcome measures representing various levels of outcome assessment. The

secondary objective is to determine if and which diagnostics will be ultimately used in future trials.

Study design

Prospective longitudinal study.

Study burden and risks

The online questionnaires will take about 30 minutes per year. The 100 patients will yearly visit the SFN expertise center during 3 years. The diagnostics will take 90 minutes per visit including a skin biopsy, carrying very low risk of infection or persistent bleeding. SFN patients will get insight into the natural course of their own disease. This can be helpful in daily living, in participation and for coping. In addition, the assessment of various outcome measures, including patient reported outcomes, gives the patient the possibility to express their experience regarding impact of their illness on their daily living and quality of life. The registry will facilitate in trial design and recruitment, since information will be gathered regarding the most sensitive metrics to be used.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)

Inclusion criteria

Definite small fiber neuropathy (SFN):
a) Subjects of 18 years and older
b) Clinical symptoms and signs of SFN
c) Abnormal intraepidermal nerve fiber density (IENFD) in skin biopsy of the distal leg
d) Subjects must give informed consent by signing and dating an informed consent form

Probable SFN:
a) Subjects of 18 years and older
b) Clinical symptoms and signs of SFN
c) Normal IENFD in skin biopsy of the distal leg
d) Abnormal quantitative sensory testing (QST)
e) Subjects must give informed consent by signing and dating an informed consent form

Exclusion criteria

a) Signs of large nerve fiber neuropathy in neurological examination (weakness, loss of vibration sense, hypo-/areflexia)
b) Abnormal nerve conduction studies

Study design

Design

Study type: Observational invasive

Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Other

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	27-08-2024
Enrollment:	100
Type:	Actual

Ethics review

Approved WMO Date:	31-05-2023
Application type:	First submission
Review commission:	METC academisch ziekenhuis Maastricht/Universiteit Maastricht, METC azM/UM (Maastricht)
Approved WMO Date:	19-09-2023
Application type:	Amendment
Review commission:	METC academisch ziekenhuis Maastricht/Universiteit Maastricht, METC azM/UM (Maastricht)

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	NL83155.068.22