Optimizing INITIation of non invasive ventilation in ALS patients

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The primary objective of this study is to build a predictive model in which we can identify which (pheno) type of ALS patients has the most benefit from NIV in improving or maintaining quality of life.

Ethical review	Approved WMO
Status	Recruiting
Health condition type	Neuromuscular disorders
Study type	Observational non invasive

Summary

ID

NL-OMON54861

Source
ToetsingOnline

Brief title
INITIALS

Condition

- Neuromuscular disorders

Synonym
Amyotrophic Lateral Sclerosis, Motor neuron disease

Research involving
Human

Sponsors and support

Primary sponsor: Universitair Medisch Centrum Groningen
Source(s) of monetary or material Support: ALS stichting

Intervention

Keyword: amyotrophic lateral sclerose, motor neuron disease, non invasive ventilation, quality of life

Outcome measures

Primary outcome

Primary study outcome is quality of life based on questionairre ALSAQ-40

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can identify which (pheno) type of ALS patients has the most benefit from NIV

in improving or maintaining quality of life.

Secondary outcome

-Determine the opinion of ALS patients who started NIV about the quality of the

preparation prior to the initiation of NIV, the timing of the initiation of NIV

and the effect of NIV.

- Determine the reasons for not starting NIV or ending NIV.

- Determine the effect of NIV on survival in patients with different ALS

phenotypes.

- Determine how QoL changes over time in both cohort 1 and 2

Study description

Background summary

Amyotrofic Lateral Sclerosis (ALS) is a disease with a downward slope with respiratory insufficiency as primary cause of death. Chronic non-invasive ventilation (NIV) is being suggested to most ALS patients in our country , however only 1/3 will actually start with it. Although some people do benefit, in other patients the benefits are less clear. However it is hard to detect benefit of the ventilatory support since the disease on its own is progressive and will further impair the patient. The question arises why chronic

ventilatory support in ALS is provided on routinely base. The reason for this is based on 1 randomized controlled trial including only 11 patients showing a prolonged survival and a better preserved quality of life (QoL) in those with a preserved bulbar function. However, another study showed that even in patients with a bulbar dysfunction a survival benefit can be obtained. Despite this it was recently showed in an uncontrolled design that the benefit of chronic NIV might be less positive. The latter might be due to the fact that patients started in this study group later, i.e. this patients had a lower vital capacity and higher PaCO2 compared to the RCT.
In summary the clinical value of chronic NIV can be debated, there is only moderate evidence for it, we do not know which (pheno)type of ALS patient is the best candidate for, while the time of initiation may be crucial.
Furthermore the international recommendations when to start NIV in ALS patients from different guidelines are expert-based, their advices are various and do not take the different characteristics/ phenotypes of the ALS patients into account
Based on these uncertainties the field is divided for knowledge and appreciation and different opinions about chronic non-invasive ventilation exist both in professionals and patients.

Study objective

The primary objective of this study is to build a predictive model in which we can identify which (pheno) type of ALS patients has the most benefit from NIV in improving or maintaining quality of life.

Study design

Design: multi-centre prospective cohort study
Duration: The total duration of the study: 3,5 years. Inclusion period: 30 months. Follow up time : 9-1,5 months. Thereafter, 6 months will be used for data analysis.
Setting: Involvement of all (4) HMV centres in the Netherlands.
Procedure:
During the first regular visit to the HMV centre patients will be asked to participate in the study. Data will be recorded during regular visits to the HMV centre. As part of the informed consent patients will be asked their permission to retrieve the following clinical characteristics from their medical record (from neurologist or rehabilitation specialist):
• Date of diagnosis
• Type of ALS at onset
• ALSFRS-R at the time of diagnosis
• Pulmonary function at the time of diagnosis
• Cognitive status (ECAS, ALS-FTD-Q) at onset
• Medical history

After informed consent is obtained the following data will be recorded:
First visit:
• Patient characteristics
• Questionnaires
• Pulmonary function

After the first visit patients will be allocated to one of the two cohorts:
• Cohort 1: patients who start NIV
• Cohort 2: patients who do not start NIV

Cohort 1:
Data will be record after 3,6 and 9 months.
• Patient characteristics
• Questionnaires
• Pulmonary function
8 Patients and 8 carers will be asked to take part in an interview

Cohort 2:
Data will be recorded every 3 months until initiation of NIV or death or max during 1,5 years after the first visit to the HMV:
• Patient characteristics
• Questionnaires
• Pulmonary function
If a patient in cohort 2 will start with NIV, the patient will be transferred to cohort 1.

Study burden and risks

There are no significant risks involved in taking part in this study. No risks are related to the medical consultation assessments. The questionnaires may cause some fatigue or discomfort. The respiratory measurements (spirometry, blood gas and transcutaneous nocturnal gasexchange by SENTEC) are usual care. We expect that only a very small number of patients will be asked to visit the HMV centre more often than usual care.
There is no treatment for ALS and care is palliative. We hope that this research will improve the care for ALS patients in relation to non-invasive ventilation. Given the devastating nature of ALS we believe that the potential benefit of the research justifies the minimal risks and burden in this study.

Contacts

Public
Universitair Medisch Centrum Groningen

Hanzeplein 1
Groningen 9713 GZ
NL
Scientific
Universitair Medisch Centrum Groningen

Hanzeplein 1
Groningen 9713 GZ
NL

Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

Age > 18 years
Patients with diagnosis of ALS, PLS or PSMA visiting the HMC centre for the first time
Ability to give informed consent.
Ability to fill in the questionnaires independently or with assistance of a caregiver.

Exclusion criteria

Use of non invasive ventilation or invasive ventilation (tracheostomy) at time of first visit to a HMV centre.
Pregnancy

Study design

Design

Study type: Observational non invasive

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

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	01-07-2022
Enrollment:	250
Type:	Actual

Medical products/devices used

Registration:	No

Ethics review

Approved WMO Date:	08-03-2022
Application type:	First submission
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO Date:	07-12-2022
Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO Date:	24-07-2023
Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO Date:	02-04-2024

Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)

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
ClinicalTrials.gov	NCT05033951
CCMO	NL73166.042.21