Development of a remote, patient-centric, clinical trial model using digital healthcare technology

Published: 23-08-2022
Last updated: 19-04-2025

Primary objective: To assess the feasibility and adherence of home-based monitoring of multiple aspects of disease progression in patients with ALS in order to develop a widely supported set of reliable digital parameters to capture data, collected...

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

Summary

ID

NL-OMON53924

Source
ToetsingOnline

Brief title
TRICALS-ORIGIN

Condition

- Neuromuscular disorders

Synonym
ALS, Amyotrofic Lateral Sclerosis

Research involving
Human

Sponsors and support

Primary sponsor: Universitair Medisch Centrum Utrecht
Source(s) of monetary or material Support: Stichting ALS Nederland

Intervention

Keyword: Amyotrophic Lateral Sclerosis, Digital biomarkers, Remote monitoring, Self-assessment

Outcome measures

Primary outcome

Patient adherence defined as the number of completed measurements divided by the total number of assessments. Adherence will be assessed per parameter.

Secondary outcome

Overall survival, defined as time to death from any cause or respiratory insufficiency (RI; defined as tracheostomy or the use of non-invasive ventilation for >22 h per day for >=10 consecutive days). Daily functioning, defined as mean change from baseline in ALSFRS-R total score and PRO-ALS score. Respiratory function, defined as mean change from baseline in VC (% predicted of normal according to the GLI-2012 reference standard) as measured by spirometry, and MND-DS. Physical function, defined as mean change from baseline in vertical variation as assessed by accelerometry. Bulbar function, defined as mean change from baseline in acoustic measures (e.g., rate, duration, voicing) and visual measures (e.g., higher order statistics of the jaw and lip) as assessed by audio-visual speech measures. Gastrostomy placement, defined as time-to-gastrostomy since enrollment. Non-invasive ventilation use, defined as time-to-non-invasive ventilation initiation since enrollment. Body weight, defined as mean change from baseline in kilograms. Clinical disease stage, defined as mean time spent in each stage of the King*s and ALS Milano-Torino staging systems. Patient burden and user-experience as assessed by custom-made

questionnaire. Patient reported outcomes measures as assessed by global

assessment ratings in a custom-made questionnaire.

Study description

Background summary

Amyotrophic lateral sclerosis (ALS) is a debilitating disease, making trial participation particular challenging. Implementing a remote, home-based monitoring infrastructure to assess trial outcomes and drug safety could make in-clinic visits superfluous, significantly lower the trial burden, and pave the way to a patient-centric clinical trial model.

Study objective

Primary objective:
To assess the feasibility and adherence of home-based monitoring of multiple aspects of disease progression in patients with ALS in order to develop a widely supported set of reliable digital parameters to capture data, collected under free-living conditions, that reflect patient-centric measures, and can be used to develop effective therapies.

Key secondary objectives:
To assess the discriminative value of remote digital endpoints in distinguishing different subtypes of motor neuron disease (i.e. fast versus slow progressing patients)

To assess the predictive value of remote digital endpoints for clinically relevant outcomes (e.g. death, wheelchair dependency or respiratory insufficiency)

To assess the validity of unsupervised, home-based vital capacity assessment versus supervised in-clinic assessment.

Estimate the Minimal Important Difference (MID) of each digital parameter

Study design

Longitudinal cohort study conducted in the UMC Utrecht with a 12-month follow-up period.

Study burden and risks

All procedures in this study are non-invasive and there is no direct physiological discomfort associated with participation. Patients are followed-up at monthly intervals and evaluated digitally at home for body weight, spirometry, accelerometery, audio-visual speech measures, and clinical progression. At baseline visit and at month 3 (at the patients* home or in-clinic) a healthcare professional will supervise the patients* spirometry testing. After completion or premature ending of the study, a close-out visit (at the patients' home or in-clinic) will be scheduled. We estimated that study participation takes approximately 13 hours distributed over a 12-month time period. Development of reliable validated technological parameters is paramount to lower trial burden, and may advance the search for effective treatment. Given the low risk and burden of this study, we believe that the benefits outweigh the risks.

Contacts

Public
Universitair Medisch Centrum Utrecht

Heidelberglaan 100
Utrecht 3584 CX
NL
Scientific
Universitair Medisch Centrum Utrecht

Heidelberglaan 100
Utrecht 3584 CX
NL

Trial sites

Listed location countries

Netherlands

Eligibility criteria

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

Inclusion criteria

1. Diagnosis with ALS according to the Gold Coast criteria
2. ENCALS risk profile ranging between -6 and -2
3. 18 years or older
4. Able to provide informed consent

Exclusion criteria

Fulfilling the criteria for respiratory insufficiency (non-invasive ventilation use >22 hours per day for 10 consecutive days or having a tracheostomy)

Study design

Design

Study type: Observational non invasive

Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Health services research

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	27-01-2023
Enrollment:	100
Type:	Actual

Ethics review

Approved WMO

Date:	23-08-2022
Application type:	First submission
Review commission:	METC NedMec

Approved WMO

Date:	16-06-2023
Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Utrecht (Utrecht)
Approved WMO Date:	27-03-2025
Application type:	Amendment
Review commission:	METC NedMec

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	NL79677.041.22