Feeding and Swallowing Problems in Spinal Muscular Atrophy (SMA). 'Eet SMAkelijk'
A Crossectional Diagnostic Cohort study to investigate mastication and swallowing disorders in Dutch patients with Spinal Muscular Atrophy type II, III and IV

Published: 30-05-2017
Last updated: 12-04-2024

To explore the nature and consequences of feeding and swallowing problems in patients with SMA type II, III, and IV, and to detect predictors of these problems from instrumental, clinical dysphagia assessments and the LINMA(-k) (an adapted...

Ethical review	Not approved
Status	Will not start
Health condition type	Neuromuscular disorders
Study type	Observational invasive

Summary

ID

NL-OMON45438

Source
ToetsingOnline

Brief title
Feeding and Swallowing Problems in Spinal Muscular Atrophy (SMA)

Condition

- Neuromuscular disorders

Synonym
Dysphagia, Swallowing disorders

Research involving
Human

Sponsors and support

Primary sponsor: Universitair Medisch Centrum Utrecht
Source(s) of monetary or material Support: Prinses beatrixfonds

Intervention

Keyword: Dysphagia, Spinal Muscular Atrophy

Outcome measures

Primary outcome

a. VFSS: Feeding and swallowing problems are investigated in the oral transit phase, the pharyngeal phase and upper oesophageal phase of swallowing, using VFSS, with primary endpoints:

Formation of bolus pureed food (yes/no); duration of oral transit phase in liquids (sec.); piece meal deglutition (yes/no); laryngeal aspiration/penetration (8-point scale);post-swallow residue in the hypopharynx (score 1-6); reduced UES opening (yes/no) and premature closure UES (yes /no).

Two independent raters will observe the video of the VFSS (15% of the total amount of VFSS, randomly chosen) and data will compared to assess interrater reliability.

b. Timed Water Swallowing test (TWST), outcome measures: ml/sec (comparisons

with normal values)(J.G.Kalf, 2011; Nathadwarawala, 1992)

Two independent raters will score this test (15% of the total amount, randomly chosen) and data will compared to assess interrater reliability.

c. Swallowing Volume Test (SVT), outcome measures: max ml water swallowed in one swallow (comparisons with normal values) (Ertekin C, 1996; J.G.Kalf, 2011).

Two independent raters will score this test (15% of the total amount, randomly chosen) and data will compared to assess interrater reliability.

d. Test for Observation of Mastication and Swallowing Solids (TOMASS), outcome measures: number of discrete bites, masticatory cycles, swallows, total time (comparisons with normal values, ML Huckabee et al).

Two independent raters will observe the video of performance of this test (15% of the total amount, randomly chosen) and data will compared to assess interrater reliability.

e. Six minute mastication test (endurance) (6MMT), outcome measures: total amount of chewing cycles and the difference between minute 1 (M1) and minute 6 (M2) (data are compared with normal values using z-scores, van den Engel-Hoek et al, 2017).

Two independent raters will observe the video of performance of this test (15% of the total amount, randomly chosen) and data will compared to assess interrater reliability.

f. Active maximum mouth opening (aMMO), outcome measures: distance between upper en lower teeth plus overbite in mm*s.

g. Ultrasound oral and facial muscles, outcome measures: echogenicity and thickness of the muscles (quantitative, comparisons with normal values, z-scores); Heckmatt -score (qualitative, score 1 = normal * score 4 = severely increased echogenicity with absent bone reflection); presence of fasciculations (yes / no) (Heckmatt, 1982).

Two independent raters will perform the analysis of the ultrasound images (both quantitative and qualitative) (15% of the total amount, randomly chosen) and data will compared to assess interrater reliability.

h. LINMA(-k), filled in by the patients (same as used in the Prevalence study, METC protocol no. 09-307), amendment 11: *Swallowing function in SMA*) in terms of no problems, mastication problems, swallowing problems or mastication and swallowing problems by the patients.

i. Oral motor abilities: a qualitative description of the oral motor abilities on the basis of the Radboud Oraal Onderzoek (qualitative scores for movements

of face, lips, tongue in terms of 0 = deviant or impossible to perform, 1 =

clearly deviant, 2 = minimally deviant or doubt, 3 = normal)

Two independent raters will observe the video of performance of this test (15%

of the total amount, randomly chosen) and data will compared to assess

interrater reliability.

Secondary outcome

Not applicable

Study description

Background summary

Spinal Muscular Atrophy (SMA) is an autosomal recessive motor neuron disease, characterized by proximal and axial muscle weakness (Lefebvre et al., 1995). Involvement of lower and middle brain stem motor nuclei is relatively common and the resulting bulbar muscle weakness is probably an important cause of feeding and swallowing problems. Problems with feeding and swallowing may result in complications including asphyxiation (Cichero et al., 2013), aspiration and respiratory infections
Several clinical studies have addressed the frequency of feeding and swallowing difficulties in patients with SMA, in particular types 2 and 3. Importantly, these studies are with few notable exceptions based on questionnaires (Chen et al , 2012; Messina et al., 2008) and not on the gold standard for dysphagia evaluation, i.e. a videofluoroscopic swallowing study (VFSS).Although it is not known whether questionnaire-based studies underestimate the frequency of feeding and swallowing problems in patients with SMA, this seems very likely. There are no studies that have systematically investigated the causes of bulbar complications in SMA, and the current recommendations are based on a study with only a small patient group (L van den Engel-Hoek et al., 2009).
In the present study the nature and factors underlying feeding and swallowing problems will be studied, with the gold standard for dysphagia evaluation, the VFSS, a range of dysphagia assessments, and a questionnaire, all part of an extensive clinical feeding and swallowing evaluation.

Study objective

To explore the nature and consequences of feeding and swallowing problems in patients with SMA type II, III, and IV, and to detect predictors of these problems from instrumental, clinical dysphagia assessments and the LINMA(-k) (an adapted questionnaire specially developed for neuromuscular diseases) with the aim to identify feeding and swallowing problems at an early stage and to develop symptomatic treatment strategies.
Secondary Objective is to establish a package of clinical feeding and swallowing assessments which is sensitive to detect feeding and swallowing problems in SMA.

Study design

The study design is a cross-sectional diagnostic cohort study. Dysphagia will be studied with the gold standard VFSS and clinical observation tools in a large group of patients with SMA II, IIIa, IIIb and IV (N=50).

Inclusion of the patients will be from June 2017 to June 2018. The study will be conducted in the UMC Utrecht.

Patients (12 years and older) from our national database *Spinal muscular atrophy, SMN protein and genetics* (dossier no. NL29692.041.09, METC protocol no. 09-307, UMC Utrecht) will be informed on the study and will be asked to consider participating in the study. After permission they are asked to come to the hospital for half a day. They fill out the questionnaire LINMA(-k) with 40 questions, (specially designed for patients with neuromuscular diseases) and they undergo instrumental dysphagia assessment (VFSS) and several clinical dysphagia assessments.

Study burden and risks

Considering the suffering of patients with SMA, the burden of participants is relatively small
and this study can be classified as a study with a negligible risk. Only one visit to the
hospital (Utrecht) will be needed. The visit takes about three hours including breaks for coffee or tea. The questionnaire and clinical testing will be followed by the instrumental swallow assessment (VFSS). Patients will benefit from the assessment, because they will be informed on the outcome measures and, if applicable, the will be informed on possible adaptations or treatment.
For VFSS participants are exposed to radiation, during a restricted number of swallows (a maximum of 15 times), which is determined to minimize the exposure of radiation. Participants are given the opportunity to object against any measurement.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

1) a clinical diagnosis of SMA type II, IIIa, IIIb, IV, and a genetically confirmed homozygous SMN1 deletion
2) given oral and written informed consent when *18 years old;
3) given informed consent by the parents or legal representative and the patient in case of patients aged *12 till <18 years old;

Exclusion criteria

1) Severe SMA, defined as clinical types 0 and I;

2) Inability to meet study visits
3) Total tube feeding

Study design

Design

Study type: Observational invasive

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

Recruitment

NL

Recruitment status:	Will not start
Enrollment:	50
Type:	Anticipated

Ethics review

Not approved

Date:	30-05-2017
Application type:	First submission
Review commission:	METC Universitair Medisch Centrum Utrecht (Utrecht)

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	NL61348.041.17