Dysphagia in children with congenital myopathy, underlying mechanisms and advices for assessment and treatment

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Objective 1To describe the course and underlying mechanisms of dysphagia in children with congenital myopathy (from 6 months to 12 years)Objective 2To formulate recommendations for early detection, diagnostic assessment , treatment and advices for...

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Other condition
Study type	Observational non invasive

Summary

ID

NL-OMON42147

Source
ToetsingOnline

Brief title
Dysphagia in children with congenital myopathy

Condition

- Other condition
- Neurological disorders congenital
- Appetite and general nutritional disorders

Synonym
Dysphagia; deglutition disorder

Health condition

Slikproblemen (dysfagie)

Research involving

Human

Sponsors and support

Primary sponsor: Universitair Medisch Centrum Sint Radboud
Source(s) of monetary or material Support: Ministerie van OC&W,Subsidie aanvraag ingediend bij Fonds Nuts OHRA

Intervention

Keyword: Assessment, Dysphagia, Myopathy, Treatment

Outcome measures

Primary outcome

Part 1 of the study, healthy infants and children

- Demographic data: sex, age, length and weight

- Phase of development feeding (1 - only breast or bottle feeding; 2- breast or bottle feeding and spoon feeding (pureed food) ; 3- chewing (solid food).

- Echo intensity of the various oral muscles (with gray scale analysis, 0-244)

- Thickness of the various oral muscles (in cm)

Regression analysis will be performed to measure which variables (sex, age, height and weight) are influencing echo intensity and muscle thickness. With these equations normal values can be established. Possible deviations can be described in Z-scores (amount of standard deviations above or under normal).

Part 2 of the study, patients with a congenital myopathy

- Demographic data: sex, age, length and weight

- Functional activities based on the BSID-3 (until 2 years of age) or the Motor Function Measure scale (0-100), (measured by the physical therapist)

- Phase of development feeding (1 - only breast or bottle feeding; 2- breast or

bottle feeding and spoon feeding (pureed food) ; 3- chewing (solid food).

- (1) Observation List Spoon feeding (score 0-35); (2) outcome SOMA per function (drinking, chewing); (3) Score on the Dysphagia Disorder Survey: : 0 - no dysphagia, 1- mild dysphagia, 2 - moderate dysphagia, 3- severe dysphagia, 4 - profound dysphagia.

- Activity submental muscle group (sEMG) and nasal flow: percentage differences between thin liquid and thick liquid during swallowing in duration (in sec) and amplitude (in µV). Coordination between swallowing and breathing will be described (1- inspiration - swallow - expiration; 2 - inspiration - swallow - inspiration; 3- expiration - swallow - inspiration; 4 - otherwise)

- Data of the analysis of the videofluroscopic swallow study, based on a list of 12 signs which are related to underlying dysphagia (score yes or no)

- Echo intensity of the various oral muscles (with gray scale analysis, 0-244), expressed as Z-scores

- Thickness of the various oral muscles, expressed as Z-scores

Secondary outcome

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Study description

Background summary

The swallow-team (children) of the Radboud University Medical Centre performs assessments and gives advices to parents of children with complex swallowing disorders (dysphagia). Based on scientific research several disease specific treatments have been developed for children with neurologic disorders or syndromes. In children with neurologic conditions, like cerebral palsy (spasticity), neuromuscular diseases (muscle diseases) and metabolic diseases,

dysphagia is often reported and is influencing negatively the quality of life of the children and their parents.
To assess the specific swallowing problems in children with neuromuscular disorders measurements and analyses were performed with videofluoroscopic swallow studies (radiological swallow investigation with different consistencies of food, mixed with contrast, with observation of the oral, pharyngeal and esophageal phase of swallowing), surface EMG of the submental muscle group and quantitative muscle ultrasound. This non-invasive method was already available for skeletal muscles and was further developed by our research group for the diagnostics of oral muscles. First of all we collected normal data in children and young adults between 5 and 30 years and these data were compared with the patient group of boys and adults with Morbus Duchenne. Based on these findings we were able to develop advices for assessment and management of dysphagia in children with spinal muscular atrophy type (SMA) II as well as for boys with Duchenne.
In these research projects disease specific mechanisms were found causing the swallowing problems, which allows us to develop new interventions. The written interventions for these two groups can not be translated one to one for children with congenital myopathy, in which facial weakness, much more pronounced than in SMA or Duchenne, and related anatomical deviances of the mandible and maxilla, are influencing swallowing. In children with congenital myopathy severe muscle weakness is present, resulting in problems with breathing, sucking and swallowing. Furthermore, the swallowing disorders in children with congenital myopathy seemed to recover slightly during the first years. Therefore, assessment, advices and possible interventions in the early years are essential.

Based on the above mentioned research projects this project is written to develop also for children with a congenital myopathy disease specific assessments and treatment protocols.
To provide parents of children with a congenital myopathy effective and preventive advices, and interventions, this research is needed to investigate the course and underlying pathological mechanisms of dysphagia in this patient group.

Study objective

Objective 1
To describe the course and underlying mechanisms of dysphagia in children with congenital myopathy (from 6 months to 12 years)
Objective 2
To formulate recommendations for early detection, diagnostic assessment , treatment and advices for dysphagia in children with congenital myopathy, as well as for involved health care professionals as for the parents of these children. A summary will be published on a fact sheet.

Study design

Part 1 of the study: Collecting normal data of echo intensity and thickness of the digastrics muscles (left and right), geniohyoid muscle, masseter muscle, temporal muscle and tongue thickness with quantitative muscle ultrasound in healthy children between 6 months and 5 years (from January 2015 to May 2015)

This is a descriptive design in which sex, age, length and weight are used to formulate normal values. These data will be related to feeding (drinking form breast or bottle, eating from a spoon, chewing solid food).

Part 2 of the study: Describing the dysphagia in 10-12 (young) children with a congenital myopathy, who are referred to the swallow team - children of the Radboudumc, or children with a congenital myopathy and feeding and swallowing problems, that are reported by their parents, after reading the announcement of this research project on the website of Spierziekten Nederland (from January 2015 to June 2016).

This is a descriptive design (cross sectional) in which the dysphagia in children with congenital myopathy will be assessed with the following assessments: observation of eating and / or drinking, questionnaire on functional activities during eating and drinking, a swallowing assessment with sEMG measurements of the submental muscle group and registration of nasal flow, and if necessary (in case of doubt about safety of the swallow act) en videofluoroscopic swallow study. Furthermore, the ultrasound measurements, described in part 1 of the study, will be performed.

Study burden and risks

There wiil be minimal risk, because the measurements are not invasive, not long standing and will be performed only once. The risks for the group (children) are negigible and the burden is minimal. The assessment will be done in presence of the parents, who will be informed.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Children (2-11 years)

Inclusion criteria

Healthy children, part 1 of the study:
- Age between 6 months and 5 years
- Healthy children with age appropriate growth;Patients, part 2 of the study:
- Diagnosed with congenital myopathy from 6 months until 12 years of age (confirmed or a not yet totally classified myopathy)
- Known with dysphagia or feeding problems

Exclusion criteria

Healthy children, part 1 of the study:
- Dysphagia
- Feeding problems, requiring tube feeding
- Neuromuscular disorder
- Diagnosis of genetic syndrome;Patients, part 2 of the study:
- Totally on tube feeding from birth with no oral feeding experience

Study design

Design

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

Primary purpose: Diagnostic

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	11-04-2017
Enrollment:	72
Type:	Actual

Ethics review

Approved WMO Date:	16-12-2014
Application type:	First submission
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	30-08-2017
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)

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	NL51506.091.14