Advanced respiratory muscle ultrasound in patients with a congenital myopathy/muscular dystrophy

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Phase 1: Assess reliability of advanced respiratory muscle ultrasound imaging in healthy subjects and congenital myopathies/muscular dystrophies. Phase 2: assess two year follow-up of respiratory function using advanced respiratory muscle ultrasound...

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

Summary

ID

NL-OMON51064

Source
ToetsingOnline

Brief title
Advanced respiratory muscle ultrasound

Condition

- Muscle disorders
- Neuromuscular disorders

Synonym
congenital muscle disorders, congenital myopathies/muscular dystrophies

Research involving
Human

Sponsors and support

Primary sponsor: Radboud Universitair Medisch Centrum
Source(s) of monetary or material Support: ZonMw

Intervention

Keyword: neuromuscular disorders, reliability, respiratory muscle ultrasound, respiratory muscle weakness

Outcome measures

Primary outcome

Phase 1: inter-rater, intra-rater and test-retest reliability in healthy

subjects and patients of advanced respiratory muscle ultrasound outcomes.

Phase 2: two year follow-up of respiratory function using advanced respiratory

muscle ultrasound in congenital myopathies/muscular dystrophies.

Secondary outcome

phase 1: validity in advanced respiratory muscle ultraound in healthy subjects

phase 2: differences between patients and healthy subjects with advanced

respiratory muscle ultrasound

Study description

Background summary

respiratory muscle weakness is a cardinal feature in many neuromuscular disorders, including congenital myopathies and congenital muscular dystrophies. Evaluating respiratory function is of vital importance to identify early signs of respiratory insufficiency, to monitor disease progression, and above all to guide respiratory management. However, reliable tools that specifically and noninvasively measure respiratory muscle function are lacking. In the last decade, ultrasound emerged as a research tool to noninvasively evaluate respiratory muscle function in patients with a neuromuscular disorders. However, it comes with limitations, such as poor clinical reliability and limited reflection of respiratory muscle function. New, advanced techniques have been developed to address these shortcomings, but have to be evaluated first.

Study objective

Phase 1: Assess reliability of advanced respiratory muscle ultrasound imaging in healthy subjects and congenital myopathies/muscular dystrophies.
Phase 2: assess two year follow-up of respiratory function using advanced respiratory muscle ultrasound imaging in congenital myopathies/muscular dystrophies.

Study design

Prospective cohort study, consisting of two phases. During phase 1, reliability of advanced respiratory muscle ultrasound will be evaluated in healthy subjects and patients. Additionally, validity will be assessed in healthy subjects. During phase 2, progression of respiratory muscle weakness in patients is evaluated, in relation to skeletal muscle weakness.

Study burden and risks

the risk of this study for the participants is negligible. Subjects do not directly benefit from participating in this study. The scientific benefit of this study is the availability of reliable and valid ultrasound measurements of the respiratory muscles along with their associated normative values and differences in disease. This can be used in subsequent research, such as drug-evaluating trials, to act as outcome parameters. Furthermore, in future clinical practice, these measurements may be used to aid diagnosis and management of respiratory muscle weakness. Respiratory functions tests may be experienced as mildly unpleasant, and the invasive procedure of placing a nasogastric tube, only in adult healthy subjects, may be experienced as discomfortable.

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)
Children (2-11 years)
Elderly (65 years and older)

Inclusion criteria

- Age:
o Phase 1: age between 18 and 60 years
o Phase 2: age between 8 and 60 years
- Healthy subjects: competent volunteers
- Patients: genetically confirmed congenital myopathy or congenital muscular dystrophy
- Willingness and ability to understand nature and content of the study
- Ability to participate and comply with study requirements

Exclusion criteria

- Medical history or current condition affecting respiratory muscle strength or function, other than a congenital myopathy or congenital muscular dystrophy (e.g. COPD).
- Active smoker
- Known upper airway / oesophageal pathology or anatomical variations of the upper airway (phase 1, healthy subjects only)
- Known bleeding disorders or frequent nasal bleeding (phase 1, healthy subjects only).

Study design

Design

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

Primary purpose: Other

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	23-12-2021
Enrollment:	122
Type:	Actual

Ethics review

Approved WMO

Date:	12-05-2021
Application type:	First submission
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

ID: 23911
Source: NTR
Title:

In other registers

Register	ID
CCMO	NL76526.091.21
Other	NL9207
OMON	NL-OMON23911