Advanced respiratory muscle ultrasound in health and congenital myopathy/muscular dystrophy

No registrations found.

Ethical review	Not applicable
Status	Pending
Health condition type	-
Study type	Observational non invasive

Summary

ID

NL-OMON23911

Source
NTR

Brief title
Advanced respiratory muscle ultrasound

Health condition

Congenital myopathies and congenital muscular dystrophies

Sponsors and support

Primary sponsor: Radboudumc
Source(s) of monetary or material Support: ZonMw Vici

Intervention

Outcome measures

Primary outcome

Phase 1: inter-rater, intra-rater and test-retest reliability in healthy subjects and patients of the following measurements:
- End-expiratory thickness, end-inspiratory thickness and thickening ratio of all respiratory

muscles during respiration
- Strain and strain rate of all respiratory muscles during respiration
- Echogenicity of all respiratory muscles during respiration
- Tissue stiffness expressed as Young’s modulus of all respiratory muscles during respiration
Phase 2: two year follow-up of respiratory function using advanced respiratory muscle ultrasound in congenital myopathies/muscular dystrophies

Secondary outcome

Phase 1: validity of advanced respiratory muscle ultrasound, as assessed by the correlation of all measurements to transdiaphragmatic pressure and surface EMG
Phase 2: differences between healthy subjects and patients of advanced respiratory muscle ultrasound

Study description

Background summary

Rationale: 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 techniques have been developed to address these shortcomings, but have to be evaluated first. Therefore, this study is aimed at evaluating new, advanced respiratory muscle ultrasound by assessing reliability, validity, normative values and disease progression in healthy subjects and patients with a congenital myopathy or congenital muscular dystrophy.

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, differences between healthy subjects and patients will be assessed and disease progression of patients is evaluated.

Study population: 61 healthy subjects and 61 patients will be included

Nature and extent of the burden, risks associated with participation, benefit and group relatedness: 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 clinical practice, these measurements may be used to aid diagnosis and management of respiratory muscle weakness. Respiratory functions tests may be experienced as mild unpleasant, and the invasive procedure of placing a nasogastric tube in healthy subjects only may be experienced as discomfortable.

Study objective

Advanced respiratory muscle ultrasound is a reliable tool to assess respiratory muscle weakness in congenital myopathies/muscular dystrophies

Study design

Phase 1: all participants, healthy subjects as well as patients, will visit the study site twice within two weeks.
Phase 2: healthy subjects will visit the study site once, while patients will visit the study site three times with one year intervals.

Intervention

None

Contacts

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Eligibility criteria

Inclusion criteria

- Informed consent from participant or legal representative
- 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 non invasive
Intervention model:	Parallel
Allocation:	Non-randomized controlled trial
Masking:	Open (masking not used)
Control:	N/A , unknown

Recruitment

NL

Recruitment status:	Pending
Start date (anticipated):	01-05-2021
Enrollment:	122
Type:	Anticipated

IPD sharing statement

Plan to share IPD: Undecided

Plan description
Not applicable

Ethics review

Not applicable
Application type: Not applicable

Study registrations

Followed up by the following (possibly more current) registration

ID: 51064
Bron: ToetsingOnline
Titel:

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
NTR-new	NL9207
CCMO	NL76526.091.21
OMON	NL-OMON51064

Study results

Summary results
Not applicable