Ultrasound imaging as a new tool for assessment of respiratory muscle structure and function in patients with Duchenne Muscular Dystrophy

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Primary objective: To quantify the degree of respiratory muscle degeneration and dysfunction using ultrasound in patients with DMD over a wide age range; from early childhood to adults.Secondary objective: To determine the association between...

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

Summary

ID

NL-OMON56724

Source
ToetsingOnline

Brief title
RESMUS-DMD

Condition

- Muscle disorders

Synonym
Duchenne disease, Duchenne Muscular Dystrophy

Research involving
Human

Sponsors and support

Primary sponsor: Radboud Universitair Medisch Centrum

Source(s) of monetary or material Support: Duchenne Parents Project

Intervention

Keyword: Duchenne, muscles, respiratory, ultrasound

Outcome measures

Primary outcome

• Respiratory muscle thickness: Thickness of the diaphragm, accessory inspiratory muscles and expiratory muscles will be examined with ultrasound

• Diaphragm excursion: Excursion of the diaphragm will be assessed

• Respiratory muscle echogenicity: quantitative muscle ultrasound (QMUS) will assess echogenicity of the diaphragm, accessory inspiratory muscles and expiratory muscles.

Secondary outcome

• All participants; Age, length, weight, medication.

• Patients: Age at disease onset, age at diagnosis

• Pulmonary function testing (PFT): forced vital capacity (FVC), Forced expiratory volume in one second (FEV1), maximal inspiratory pressure (MIP), maximal expiratory pressure (MEP) and sniff nasal inspiratory pressure (SNIP).
For patients, the PFT will be assessed by a pediatric rehabilitation doctor.
For healthy participants, it will be assessed by a researcher.

• Clinical history: A standardized clinical history will be assessed, including documentation of use of steroids and respiratory support, airway infections and degree of scoliosis.

Study description

Background summary

Duchenne muscular dystrophy (DMD) is a progressive neuromuscular disease, which also includes respiratory muscle weakness, resulting in respiratory failure and life-threatening pulmonary infections. Therefore, respiratory outcome measures are of vital importance to guide respiratory management and evaluate novel therapies in clinical trials. Currently, spirometry is frequently used to assess pulmonary function. However, this is not a direct measurement of respiratory muscle function, and is difficult to perform for young children. Recently, the applicant evaluated advanced ultrasound techniques and demonstrated that these techniques provide reliable outcome measures on respiratory muscle structure and function. Quantitative muscle ultrasound (echogenicity) detects structural changes due to fat infiltration or fibrous tissue, whilst conventional muscle ultrasound (thickening, excursion) detects respiratory muscle dysfunction.

Study objective

Primary objective: To quantify the degree of respiratory muscle degeneration and dysfunction using ultrasound in patients with DMD over a wide age range; from early childhood to adults.
Secondary objective: To determine the association between respiratory muscle ultrasound measures and pulmonary function in DMD patients and healthy subjects over a wide age range

Study design

The proposed study is an cross-sectional study in patients with Duchenne Muscular Dystrophy (DMD) and healthy age-matched subjects. The measurements will be performed at the Amalia Children*s Hospital at Radboudumc in Nijmegen, the Netherlands. The measurements will take 30 minutes for patients and 1 hour for healthy participants. The visitations for patients will be scheduled following other visitations at Radboudumc. DMD patients come to Radboudumc one time per year, to minimize the burden of travelling to Nijmegen multiple times. Therefore the schedule is very full. The measurements for this study will be performed directly after logopedics, who also use the ultrasound device. This minimizes the duration before starting of the measurements. Four muscle groups will be measured:
1. Diaphragm (both subcostal and intercostal approach)
2. Abdominal wall muscles (internal oblique, external oblique, transverse abdominis)
3. Parasternal muscles
4. Sternocleidomastoid.

Study burden and risks

The risk of this study for the participants is negligible, according to the NFU risk classification. Subjects will not directly benefit from participating in this study. The scientific benefit of this study is find outcome measures for degeneration and function of respiratory muscles in DMD patients, which can possibly be used as outcome measures in future clinical trials.
Patients will continue their medication as usual. This prevents patients from experiencing worsening of symptoms caused by participation of the study.

The pulmonary function test may be experienced as mildly unpleasant. However, the patients need to perform pulmonary function testing as regular care. Therefore, the relatively small burden of the PFT will only be applicable for healthy participants.

The burden of the ultrasound is relatively small. The participants are asked to perform tidal breathing and maximal inspiratory breathing. The duration of the ultrasound will probably not be experienced as unpleasant. There are no invasive procedures in this study.

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)

Inclusion criteria

Healthy subjects: competent healthy male volunteers.
Patients: - Patients with genetically proven Duchenne Muscular Dystrophy who are currently receiving care at Radboudumc.

All participants: - Ability to participate and comply with requirements
- If >16: willingness and ability to understand nature and content of the study
- If 12-16: Parents and participant with willingness and ability to understand nature and content of the study.
- If <12: Parents with willingness and ability to understand nature and content of the study.

Exclusion criteria

Healthy volunteers: - Previous or ongoing neuromuscular disorders.
- Previous or ongoing pulmonary deficiencies.
- Smoking
Patients: - Chronic lung, cardiac or liver disease
- Additional diagnosed lung disease

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:	Recruiting
Start date (anticipated):	06-05-2024
Enrollment:	90
Type:	Actual

Ethics review

Approved WMO Date:	03-04-2024
Application type:	First submission
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	24-12-2024
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	NL85831.091.23