Respiratory muscle training in patients with Spinal Muscular Atrophy

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The aim of this study is to assess the feasibility and efficacy of respiratory muscle training (RMT) in patients with SMA and respiratory muscle weakness.

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

Summary

ID

NL-OMON52847

Source
ToetsingOnline

Brief title
RESISTANT

Condition

- Neurological disorders congenital
- Congenital respiratory tract disorders

Synonym
Spinal Atrophy, Spinal Muscular Atrophy

Research involving
Human

Sponsors and support

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

Intervention

Keyword: respiratory muscle, SMA, Spinal muscular atrophy, training

Outcome measures

Primary outcome

We hypothesize that an individualized incremental home based respiratory muscle training program will be feasible (good adherence and good acceptability) and will improve inspiratory and expiratory muscle strength (primary outcome measure) and patient reported breathing difficulties, the onset of respiratory infections and health related quality of life (secondary outcome measure) in patients with SMA.

Secondary outcome

Reporterd airway problems by patients

Number of airway infections

Health related quality of life.

Evaluation experience trainingsperiod.

Study description

Background summary

Spinal Muscular atrophy (SMA) is characterised by progressive and predominantly proximal and axial muscle atrophy and weakness. Weakness of the respiratory muscles results in nocturnal hypoventilation, weak cough and ultimately respiratory failure in the most severely affected patients, which can lead to premature death. Treatment strategies that slow down the decline or improve respiratory muscle function are therefore needed.

Study objective

The aim of this study is to assess the feasibility and efficacy of respiratory

muscle training (RMT) in patients with SMA and respiratory muscle weakness.

Study design

The effect of RMT will be investigated with a single blinded randomized sham-controlled cross over trial consisting of a 4 months training period followed by 4 months of sham-controlled training

Study burden and risks

The risks of the tests and training are negligible. Similar tests and training methods used in usual care are well tolerated in other patients groups with neuromuscular disorders. *
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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

* Patients with SMA aged >= 8 years with respiratory muscle weakness (Maximal inspiratory pressure < -80 cmH2O) will be invited to participate.
Both patients without respiratory support and patients on night-time non-invasive ventilatory support are eligible.
* Patients who are treated with Spinraza® for more than 2 months and patients who are not/or will not be treated with Spinraza® .

Exclusion criteria

* tracheostomy,
* inability to perform respiratory and/or lung-function testing,
* inability to understand Dutch or English,
* patients who are mentally incompetent,
* patients with a history of pneumothorax or symptomatic low cardiac output syndrome
* patients who start Spinraza during trial

Study design

Design

Study type:	Observational non invasive
Intervention model:	Parallel
Allocation:	Randomized controlled trial
Masking:	Single blinded (masking used)
Control:	Placebo
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	02-02-2021

Enrollment:	50
Type:	Actual

Ethics review

Approved WMO Date:	10-08-2020
Application type:	First submission
Review commission:	METC NedMec
Approved WMO Date:	18-06-2021
Application type:	Amendment
Review commission:	METC NedMec
Approved WMO Date:	15-09-2022
Application type:	Amendment
Review commission:	METC NedMec
Approved WMO Date:	02-06-2023
Application type:	Amendment
Review commission:	METC NedMec

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	NL73280.041.20