Spinal Muscular Atrophy Randomized Controlled Trial on Cough Techniques (Patient tailored airway clearance in patients with Spinal Muscular Atrophy: a randomized controlled trial comparing air stacking and mechanical insufflation-exsufflation.)

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The aim of this study is to compare the effect of twice daily MI-E to twice daily AS with MAC on increase of PECF in patients with SMA with weak cough over a period of 3 years.

Ethical review	Approved WMO
Status	Recruiting
Health condition type	Musculoskeletal and connective tissue disorders congenital
Study type	Interventional

Summary

ID

NL-OMON56529

Source
ToetsingOnline

Brief title
SMARCT

Condition

- Musculoskeletal and connective tissue disorders congenital
- Respiratory tract infections

Synonym
Spinal muscular atrophy (SMA)

Research involving
Human

Sponsors and support

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

Intervention

Keyword: Airstacking, Mechanical Insufflation-Exsufflation, Spinal Muscular Atrophy

Outcome measures

Primary outcome

Primary endpoint is the immediate effect on improvement of Peak Expiratory

Cough Flow (PECF) over a period of 3 years

Secondary outcome

Secondary endpoints are the number of respiratory tract infections (RTIs)

requiring hospital admissions or antibiotics, patient reported patient

satisfaction, lung function decline, patient satisfaction, adverse events and

compliance to treatment

Study description

Background summary

Spinal Muscular Atrophy (SMA) is a neuromuscular (NMD) with progressive respiratory muscle weakness, resulting in progressive decline of lung function, impaired cough with recurrent RTIs and finally respiratory failure. Cough impairment due to respiratory muscle weakness underlies a cycle of events that contributes to progressive lung function decline: inadequate cough results in ineffective airway clearance, leading to mucus plugging, atelectasis, and respiratory tract infections (RTIs). Recurrent RTIs lead to further respiratory muscle weakness, with a resulting vicious circle. The shortened life expectancy in SMA is primarily caused by these respiratory problems. Impaired airway clearance due to weakness of respiratory muscles is common and

represents a challenge in SMA care. Different guidelines suggest to introduce airway clearance techniques (ACTs) in patients with NMDs when Peak (Expiratory) Cough Flow (P(E)CF drops below 270 l/min. A very common way to assist both inspiration and expiration consists in combining assisted inspiration, using techniques like air stacking (AS), with manually assisted coughing (MAC). Mechanical insufflation-exsufflation (MI-E) is another technique, which combines inspiratory and expiratory aids. There is no evidence which of these ACTs increases P(E)CF most, and results in reduced number of RTIs in this vulnerable patients.

Study objective

The aim of this study is to compare the effect of twice daily MI-E to twice daily AS with MAC on increase of PECF in patients with SMA with weak cough over a period of 3 years.

Study design

Randomized controlled trial. We hypothesize a greater increase in PECF in patients treated with MI-E compared to AS with MAC

Intervention

MI-E or AS with MAC should be used at least twice a day

Study burden and risks

Patients will be visiting the UMCU Utrecht every 4 to 8 months, which will be in combination with regular visit. During this visit lung function tests will be done which are the same as during regular follow up, except from measuring PECF after performing ACT (MIE or AS with MAC). Patients are also requested to fill a diary on compliance and patient satisfaction once a month. Both AS as well as MI-E are treatments used during regular care and there are no concerns regarding safety. Inclusion of children is necessary, as compromised cough resulting in respiratory tract infections and respiratory failure, is present from early childhood. In adult patients with severe lung function restriction for many years, the compliance of the chest is diminished, resulting in less lung volume recruitment compared to children with shorter disease duration and for that reason more compliant chest.

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 Spinal Muscular Atrophy
Peak expiratory cough flow < 270 L/min if >= 10 years or PECF <200 L/min in patients 8-9 years
Patients from 8 years of age.
Able to perform spirometry

Exclusion criteria

- Severe gastroesophageal reflux with risk of aspiration despite treatment
- Severe esophageal and gastric varices
- Recent pneumothorax (< 6 weeks)
- Recent barotrauma
- Emphysema, bullae
- Tracheo-oesphageal fistula
- Severe facial deformity

- Tracheostomy
- Patient or legal representative unable to speak and understand Dutch or English
- RTI in 6 weeks prior to inclusion. If the patient suffers from a RTI in the period between screening and baseline measurements, it is not required to repeat the screening.
- Respiratory muscle training initiated < 6 weeks prior to inclusion
-Daily use of mechanical insufflation-exsufflation.Patients who used MI-E temporarily during a respiratory tract infection in the past are eligible for inclusion.*

Study design

Design

Study type:	Interventional
Intervention model:	Parallel
Allocation:	Randomized controlled trial
Masking:	Open (masking not used)
Control:	Active
Primary purpose:	Prevention

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	09-07-2024
Enrollment:	46
Type:	Actual

Medical products/devices used

Generic name:	Nippy Clearway 2
Registration:	Yes - CE intended use

Ethics review

Approved WMO

Date:	06-02-2024
Application type:	First submission
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	NL85606.041.23