ISRCTN21996121 https://doi.org/10.1186/ISRCTN21996121

# Impact of In-exsufflator Treatment on hospitalisation for Respiratory Exacerbation in Neuromuscular Disease

| Submission date | Recruitment status | [X] Prospectively registered |
|---|---|---|
| 26/06/2007 | No longer recruiting | [ ] Protocol |
| **Registration date** | **Overall study status** | [ ] Statistical analysis plan |
| 13/09/2007 | Completed | [ ] Results |
| **Last Edited** | **Condition category** | [ ] Individual participant data |
| 07/11/2007 | Nervous System Diseases | [ ] Record updated in last year |

**Plain English summary of protocol**
Not provided at time of registration

## Contact information

**Type(s)**
Scientific

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## Additional identifiers

**EudraCT/CTIS number**

**IRAS number**

**ClinicalTrials.gov number**

**Secondary identifying numbers**

N/A

# Study information

## Scientific Title

## Acronym
In-ex TREND study

## Study objectives
We will be looking specifically at a heterogeneous group of neuromuscular disorders which, either as a secondary consequence of degeneration of the spinal nerves (spinal muscular atrophy, amyotrophic lateral sclerosis) or as a primary muscle defect (muscular dystrophies, myopathies) result in progressive loss of muscle strength. Respiratory complications are the primary cause of morbidity and mortality associated with these diseases, as involvement of the respiratory muscles leads to either progressive hypoventilation or recurrent atelectasis and pneumonia secondary to decreased cough efficacy.

For this study we will look at those children with neuromuscular disorders who are admitted to hospital with a respiratory deterioration (hypoxemia and/or the presence of new onset radiologically proven atelectasis or consolidation).

Hypotheses:
We expect that the addition of the Emerson in-exsufflator to a standard treatment regimen for acute respiratory deterioration:
1. Will result in a decreased duration of hospitalisation in a population of children with neuromuscular disease
2. Resulting in hospitalisation will decrease the time requiring supplemental oxygen in a population of children with neuromuscular disease
3. Resulting in hospitalisation will result in a more rapid improvement in chest X-ray changes in a population of children with neuromuscular disease
4. Resulting in hospitalisation will decrease the length of stay in intensive care unit or days invasively ventilated in a population of children with neuromuscular disease

## Ethics approval required
Old ethics approval format

## Ethics approval(s)
Each site will submit to their local hospital ethics boards. Ethics approval received from the Children's Hospital of Eastern Ontario Research Ethics Board (REB) on the 2nd October 2007 (ref: 07/24E).

## Study design
Multi-centre randomised unblinded controlled trial of the mechanical inexsufflator. Randomisation sequence will be stratified by centre with a block-size randomisation protocol.

## Primary study design
Interventional

## Secondary study design

Randomised controlled trial

## Study setting(s)
Hospital

## Study type(s)
Treatment

## Participant information sheet

## Health condition(s) or problem(s) studied
Children with neuromuscular disorders who are admitted to hospital with a respiratory deterioration (hypoxemia and/or the presence of new onset radiologically proven atelectasis or consolidation).

## Interventions
1. Conventional treatments, as deemed appropriate by the treating physician:
1.1. Physiotherapy
1.2. Nutritional support
1.3. Antibiotics (fever, elevated White Blood Cells [WBC])
1.4. Non-invasive positive pressure ventilation
2. Conventional treatments and Emerson in-exsufflator

Using Friedman's formula for survival analysis study design, 62 patients per arm would achieve 80% power to detect a hazard ratio of 1.4. To account for potential withdrawals and withdrawal of consent, estimated at about 2.5%, four additional participants will be recruited for a grand total of 128 participants.

## Intervention Type
Other

## Phase
Not Specified

## Primary outcome measure
Time to discharge: an estimate of the primary end-point, time to discharge with standard care, was based on the clinical experience of the principal investigators and is currently being verified with a three-year retrospective chart review at Childrens Hospital of Eastern Ontario (CHEO). The mean length of stay in these patients is estimated to be 10 days. Discussions with several paediatric respirologists have taken place, focusing on what magnitude of difference in time to discharge would be clinically important between treatment and control groups. The consensus was a Minimally Clinically Important Difference (MCID) of three days' reduction from the average current length of stay in the study population. These numbers translate to a hazard ratio of 1.4. A two-sided time-to-event test at a = 0.05 will be used to detect a significant difference in time to discharge between the two arms.

## Secondary outcome measures
1. Time (in days) to improvement in oxygenation (no longer requiring supplemental oxygen for 24 hours)
2. X-ray changes: improvement or progression (increasing atelectasis, consolidation)
3. Development of acute hypercapnic respiratory failure requiring intubation and mechanical

ventilation
4. Days in intensive care unit

### Overall study start date
01/10/2007

### Completion date
01/04/2009

## Eligibility

### Key inclusion criteria
1. Patients aged 3 to 17 years
2. Patients have a known neuromotor disorder affecting respiratory muscles
3. Admitted to hospital with a respiratory deterioration (hypoxemia in the presence of new onset radiologically proven atelectasis or consolidation)

### Participant type(s)
Patient

### Age group
Child

### Lower age limit
3 Years

### Upper age limit
17 Years

### Sex
Both

### Target number of participants
128

### Key exclusion criteria
1. Refusal to participate
2. Already using the Emerson in-exsufflator at home on a regular basis
3. Development of new uncompensated hypercapnic respiratory failure requiring intubation and mechanical ventilation
4. History of bullous emphysema, known susceptibility to pneumothorax or pneumomediastinum, or known to have had any recent barotraumas

### Date of first enrolment
01/10/2007

### Date of final enrolment
01/04/2009

## Locations

### Countries of recruitment
Canada

### Study participating centre
**Children's Hospital of Eastern Ontario**
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## Sponsor information

### Organisation
Children's Hospital of Eastern Ontario (Canada)

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### Sponsor type
Hospital/treatment centre

### Website
http://www.cheo.on.ca/english/hub.shtml

### ROR
https://ror.org/05nsbhw27

## Funder(s)

### Funder type
Charity

### Funder Name
Fight Spinal Muscular Atrophy (FightSMA) (USA)

# Results and Publications

**Publication and dissemination plan**
Not provided at time of registration

**Intention to publish date**

**Individual participant data (IPD) sharing plan**

**IPD sharing plan summary**
Not provided at time of registration