

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

|                                        |                                                      |                                                              |
|----------------------------------------|------------------------------------------------------|--------------------------------------------------------------|
| <b>Submission date</b><br>26/06/2007   | <b>Recruitment status</b><br>No longer recruiting    | <input checked="" type="checkbox"/> Prospectively registered |
| <b>Registration date</b><br>13/09/2007 | <b>Overall study status</b><br>Completed             | <input type="checkbox"/> Protocol                            |
| <b>Last Edited</b><br>07/11/2007       | <b>Condition category</b><br>Nervous System Diseases | <input type="checkbox"/> Statistical analysis plan           |
|                                        |                                                      | <input type="checkbox"/> Results                             |
|                                        |                                                      | <input type="checkbox"/> Individual participant data         |
|                                        |                                                      | <input type="checkbox"/> 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

## 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).

### Primary study design

Interventional

### Study design

Multi-centre randomised unblinded controlled trial of the mechanical in-exsufflator. Randomisation sequence will be stratified by centre with a block-size randomisation protocol.

### Study type(s)

Treatment

### 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(s)**

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  $\alpha = 0.05$  will be used to detect a significant difference in time to discharge between the two arms.

## **Key secondary outcome(s)**

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

## **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

**Healthy volunteers allowed**

No

**Age group**

Child

**Lower age limit**

3 Years

**Upper age limit**

17 Years

**Sex**

All

**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

Ottawa

Canada

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**Sponsor information****Organisation**

Children's Hospital of Eastern Ontario (Canada)

**ROR**

<https://ror.org/05nsbhw27>

## **Funder(s)**

**Funder type**

Charity

**Funder Name**

Fight Spinal Muscular Atrophy (FightSMA) (USA)

## **Results and Publications**

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

**IPD sharing plan summary**

Not provided at time of registration