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Horizon Scanning Technology Prioritising Summary

CoughAssist® insufflator-exsufflator for patients with neuro-muscular disease.

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AHTA

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Enquiries about the content of the report should be directed to:

HealthPACT Secretariat
Department of Health and Ageing
MDP 106
GPO Box 9848
Canberra ACT 2606
AUSTRALIA

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This Horizon scanning prioritising summary was prepared by Linda Mundy and Professor Janet Hiller from the National Horizon Scanning Unit, Adelaide Health Technology Assessment, Discipline of Public Health, School of Population Health and Clinical Practice, Mail Drop 545, University of Adelaide, Adelaide, SA, 5005.

PRIORITISING SUMMARY

REGISTER ID: **000372 (REFERRAL)**

NAME OF TECHNOLOGY: **THE COUGHASSIST® INEXSUFFLATOR**

PURPOSE AND TARGET GROUP: **THE MECHANICAL INSUFFLATION-EXSUFFLATION TO ASSIST BREATHING IN PATIENTS WITH NEUROMUSCULAR CONDITIONS**

STAGE OF DEVELOPMENT (IN AUSTRALIA):

- ☐ Yet to emerge
- ☐ Experimental
- ☐ Investigational
- ☐ Nearly established
- ☒ Established
- ☐ Established *but* changed indication or modification of technique
- ☐ Should be taken out of use

AUSTRALIAN THERAPEUTIC GOODS ADMINISTRATION APPROVAL

- ☒ Yes — ARTG number 144321
- ☐ No
- ☐ Not applicable

INTERNATIONAL UTILISATION:

COUNTRY	LEVEL OF USE		
	Trials Underway or Completed	Limited Use	Widely Diffused
United States	✓		
Italy	✓		
France	✓		
Germany	✓		
United Kingdom	✓		

IMPACT SUMMARY:

Respironics Australia Pty Ltd provides the Cough-assist, an inexsufflator with the aim of assisting breathing in patients with neuromuscular conditions such as muscular dystrophy. The technology would be made available through public hospitals and in the domestic setting.

BACKGROUND

A normal cough requires an enlarged airway diameter and an inspiration that is between 60-80 per cent of total lung capacity. Inspiration is followed by a rapid and firm closure of the glottis. Intrathoracic pressure increases as the expiratory muscles contract, and when the glottis reopens, air is expelled and secretions are forced

towards the central airways and mouth. Inspiratory, expiratory and bulbar muscle function is necessary for an effective normal cough, however this muscle function is impaired in patients with neuromuscular disease (NMD). Complications of poor respiratory function include pneumonia, respiratory tract infection, atelectasis[1] and impaired gas exchange, which may result in patients relying on supplemental oxygen and respiratory acidosis. Respiratory failure or an inability to clear secretions from airways caused by ineffective coughing may result in an emergency tracheotomy being performed, or even death (Bach 2003; Miske et al 2004).

Airway clearance may be improved by the employment of manual or mechanical cough assistance, with or without insufflation[2]. Mechanical devices such as the CoughAssist® (Figure 1) provide insufflation-exsufflation via a face mask and promote maximum lung inflation during inspiration with the use of positive pressure. A normal cough is simulated when inflation is followed by a rapid switch to negative pressure in the upper airway (Fauroux et al 2008; Miske et al 2004). The device can also be used in patients with tracheostomy or endotracheal tubes (Mellies et al 2005).

Figure 1 The CoughAssist® mechanical insufflator-exsufflator (Respironics 2008)

CLINICAL NEED AND BURDEN OF DISEASE

Muscular Dystrophy (MD) is the name given to a group of genetic neuromuscular diseases which are characterised by the progressive weakness and degeneration of the skeletal muscles which control movement. There are a number of types of MD including Duchenne, Becker, Congenital, Limb-Girdle, FacioScapuloHumeral, Distal and Myotonic. All of the diseases under the MD umbrella vary in their pattern of inheritance, genes affected, age of onset and rate of disease progression. Australian prevalence data are not available for MD, however Access Economics conducted a meta-analysis of overseas data to estimate an Australian prevalence. There are approximately 3,457 Australians living with MD. Although overall the sex ratio of

[1] Partial or complete collapse of the lung usually due t an obstruction of the bronchus
[2] The act of breathing on or into anything, the act of blowing into any cavity of the body. Exsufflation is a strongly forced expiration of air from the lungs.

affected patients is comparable (56% male), the majority (82%) of younger sufferers (aged 0-14 years) are male indicating late onset of disease in females. The relative risk of mortality with MD is high at 424 times the population risk for males and 149 times the risk for females. It has been estimated that 290 MD sufferers died in 2005 in Australia, and of these 133 (35%) were children under the age of 15 years. Morbidity caused by MD includes pulmonary and cardiac complications as well as mental retardation (Access Economics Pty Ltd 2007).

Information on the number of individuals with muscular dystrophy living in New Zealand was difficult to obtain. In the year 2003-04, a total of 28 patients with MD were treated in public hospitals with a mean stay of 82.5 days. Again, the majority of patients were male (82%) and young (60.7% aged 0-15 years). Seven patients were treated as day cases. A total of eight patients died as a result of MD in the year 2004 (data supplied by the NZ Health Information Service).

DIFFUSION

There are approximately 50 CoughAssist® units currently in use in Australia. The majority of these units are funded by charities or associations. The Motor Neuron Association of Western Australia has committed to purchasing 30 CoughAssist® units and has already purchased 15 units. The Muscular Dystrophy Association of Victoria has eight units and Queensland have several units. The majority of units are for in-home use, however they are transported to hospitals if required (personal communication Respironics Ltd).

COMPARATORS

Manual cough assistance may not be effective in patients with a chest wall distortion from scoliosis as it is difficult to obtain an optimal hand position. In addition, this technique may not be suitable to perform in young infants or children who may have compliant chest walls (Miske et al 2004). The standard therapy for respiratory failure is non-invasive positive pressure ventilation (NIPPV) using a full facial or nasal mask to deliver ventilation support from a flow generator. NIPPV improves ventilation by unloading fatigued ventilatory muscles. NIPPV can be applied intermittently for short periods, which may be sufficient to reverse the ventilatory failure. The advantage of NPPV is that patients do not need to be sedated. The incidence of nosocomial pneumonia with NIPPV is lower than in intubated patients (Ram et al 2004). However, not all patients can be managed with NIPPV and may require tracheotomy. Failure of NIPPV may be due to the effect of retained secretions which may be cleared from airways by the CoughAssist® device (Mellies et al 2005).

SAFETY AND EFFECTIVENESS ISSUES

The small case series conducted by Fauroux et al (2008) reported on the effect of the CoughAssist® device on 17 consecutive paediatric patients with NMDs (Duchenne muscular dystrophy n=4, spinal muscular atrophy n=4, other congenital myopathy

n=9) who were in a stable state (level IV intervention evidence). Respiratory function was measured before and after treatment. Treatment consisted of three mechanical insufflation-exsufflation (MI-E) sessions, with each session comprising six cycles of insufflation-exsufflation. Each cycle consisted of two seconds insufflation followed by three seconds of exsufflation, with a 30-second rest period between each application. Inspiratory-expiratory pressures increased with each session, starting at +15 to -15 cm H_2O, followed by +30 to -30 cm H_2O with the last session +40 to -40 cm H_2O. There was a significant increase in the patient's respiratory comfort score as measured on a visual analog scale (p=0.02). There was a pressure-dependent increase in the maximal and mean inspiratory and expiratory flows ie greatest flow (L/min) was observed during MI-E application at 40cm H_2O. This resulted in a proportional increase in expiratory volume. During the 40cm H_2O the mean expiratory volume was almost twice that of the mean vital capacity[3] at baseline (1.87 ± 1.04 L vs 1.04 ± 1.13 L). The authors concluded that the CoughAssist®, which had previously only been used in an adult population, was well tolerated in a paediatric population, and resulted in short-term physiologic benefits (Fauroux et al 2008).

The retrospective study by Vianello et al (2005) compared the short-term outcomes of 11 NMD patients with upper respiratory tract infections treated with MI-E and chest physiotherapy to 16 historical NMD controls treated with physiotherapy alone (level III-3 intervention evidence). MI-E treatment consisted of five positive-negative pressure cycles followed by 20-30 seconds of normal breathing to prevent hyperventilation. Each session consisted of five or more treatments. Treatment failure was defined as the need for cricothyroid mini-tracheostomy or endotracheal intubation. The mean number of MI-E sessions per day was 2.7 ± 0.9. The mean insufflation and exsufflation pressures were 19.1 ± 3 and 33.2 ± 4.6 cm H_2O, respectively. In the MI-E group, treatment failed in 2/11 (18%) of patients compared to 10/16 (62.5%) in the physiotherapy group (p=0.047). There was no statistically significant difference between the two groups for patients requiring bronchoscopy assisted aspiration (45.5% vs37.5%, p=0.71). Patients in the MI-E group received mechanical ventilation for a mean of 9.4 ± 6.9 days compared to 13.5 ± 11.9 days for the physiotherapy alone patients. Although there was a trend towards less time spent on mechanical ventilation, due to the small numbers enrolled in this study the difference was not significant. There was no statistically significant difference between the two groups for the number of days spent in hospital (20.5 ± 20 vs 19.8 ± 17 days) or for the number of patients who received antibiotics (100% vs 81%). On the whole, treatment with MI-E was well tolerated by patients, with only two adverse events recorded. One patient experienced stomach distension at +20 to -35 cm H_2O but continued treatment. One patient developed mild nasal bleeding which resolved without specific treatment (Vianello et al 2005).

[3] Vital capacity = is the maximum volume of air that a person can exhale after maximum inhalation or the maximum volume of air that a person can inhale after maximum exhalation.

A larger retrospective study by Miske et al (2004) reported on the use of home use of MI-E in 62 NMD patients (Duchenne muscular dystrophy n=16, spinal muscular atrophy n=22, other congenital myopathy n=12, non-specific NMD n=12) (level IV intervention evidence). At initiation of therapy, 25 patients relied on non-invasive ventilation and 29 used invasive ventilation via tracheostomy. Eight patients had no ventilatory support. Treatment sessions were similar to those described above. The median age at the beginning of MI-E therapy was 11.3 years (range 3 months to 28.6 years). Mediation duration of MI-E use was 13.4 months (range 0.5 to 45.5 months), with most patients using MI-E on a daily basis. Five patients chose to discontinue use of MI-E, three of whom felt that the device was ineffective or unpleasant. Two of these patients were infants whose parents interpreted their crying as a response to the treatment. Four patients (6.5%) reported an improvement in chronic atelectasis and five (8.1%) reported a reduction in the frequency of pneumonia after the beginning of the MI-E treatment regime (Miske et al 2004).

COST IMPACT

The low prevalence of MD results in low health system expenditure, accounting for only 0.01 per cent of total health expenditure. MD is high in indirect costs due to the early onset of disease and the high mortality rate in children and as a consequence, lost productivity. In per capita terms, there is a financial cost of approximately $126,000 per person per annum, including lost productivity, the value of carers for patients, welfare payments and direct health system expenditure. The Access Economics report recommended a number of areas which should be addressed to assist sufferers of MD including early diagnosis and intervention, carer assistance and the appropriate health service delivery of interventions that address the pulmonary and cardiac complications of MD, including non-invasive ventilory support (Access Economics Pty Ltd 2007).

The CoughAssist® device costs approximately $8,500. Consumables, depending on patient needs, include the mouthpiece, facemask or connection for tracheostomy patients. In addition, a single patient use filter and circuit must be purchased, which, depending on the interface used, cost $15-$30 (personal communication Respironics Ltd).

ETHICAL, CULTURAL OR RELIGIOUS CONSIDERATIONS

No issues were identified/raised in the sources examined.

OTHER ISSUES

No issues were identified/raised in the sources examined.

SUMMARY OF FINDINGS

The use of MI-E appears to be well tolerated in both adult and paediatric patients, however there appears to be little long-term data available on the effect of MI-E on

infection and hospitalisation rates in NMD patients. The lack of good quality evidence needs to be addressed by the conduct of a randomised trial comparing MI-E to other techniques.

HealthPACT action:

Based on the low level evidence assessed in this summary, the use of non-invasive mechanical insufflation-exsufflation appears to be a useful adjunct in the management of patients with neuromuscular disease who have impaired airway function. Although long-term comparative data is lacking, this small patient group may benefit greatly from this therapy. Therefore HealthPACT recommended that this technology be monitored for further information in 12-months time.

Number of included Studies

Total number of studies	
Level IV intervention evidence	2
Level III-3 intervention evidence	1

References:

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Ram, F. S., Picot, J. et al (2004). 'Non-invasive positive pressure ventilation for treatment of respiratory failure due to exacerbations of chronic obstructive pulmonary disease', *Cochrane Database Syst Rev*, (3), CD004104.

Respironics (2008). *CoughAssist: Mechanical Insufflator-Exsufflator* [Internet]. Available from: http://coughassist.respironics.com/ [Accessed 8th May].

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Search Criteria to be used:

Cough/rehabilitation
Forced Expiratory Volume
Insufflation/*methods

Neuromuscular Diseases/*complications
Physical Therapy Modalities/methods
Positive-Pressure Respiration/*methods
Pulmonary Gas Exchange
Pulmonary Ventilation/physiology
Respiratory Function Tests
Respiratory Insufficiency/etiology/*therapy
Respiratory Muscles/*physiopathology