Outcome of noninvasive ventilation in children with neuromuscular disease

Record Status
This is a critical abstract of an economic evaluation that meets the criteria for inclusion on NHS EED. Each abstract contains a brief summary of the methods, the results and conclusions followed by a detailed critical assessment on the reliability of the study and the conclusions drawn.

Health technology
The study examined noninvasive ventilation (NIV) in children with severe neuromuscular disorders. NIV was defined as the delivery of ventilatory support via a nasal or face-mask interface without the need for an invasive artificial airway. The comparator appears to have been current practice before the introduction of NIV.

Type of intervention
Palliative care.

Economic study type
Cost-effectiveness analysis.

Study population
The study population comprised children with severe neuromuscular disorders.

Setting
The study setting was inpatient care. The economic analysis was carried out in Australia.

Dates to which data relate
The effectiveness data were collected from 1 January 1994 to 1 January 2004. The cost data were collected one year before and after study enrolment. The price year was not reported.

Link between effectiveness and cost data
The costing was performed retrospectively on those patients enrolled in the effectiveness study.

Study sample
The authors did not undertake any power calculations to determine the sample size. They decided to include in the study all patients commencing nocturnal NIV for respiratory compromise associated with neuromuscular disorders at the Children's Hospital at Westmead, Australia, for a period of 10 years. Patients with severe cognitive deficits were excluded from the QoL analysis, but were included in the remainder of the study. Seventeen patients were eligible for inclusion but 3 refused to participate or had been transferred to adults units, so 14 were enrolled (82%). Three patients commenced NIV between 1994 and 1999 and 11 patients commenced NIV between 2000 and 2004.

Study design
The authors designed a prospective within-group comparison study to be performed in a single study centre (the Children's Hospital at Westmead, Australia). Blinding was not relevant as there was no actual comparison group of patients. Data were collected pre-NIV intervention and post-NIV intervention in all patients. The median length of
follow-up was 30 months.

Analysis of effectiveness
The primary outcomes, in terms of clinical effectiveness, were patients’ symptoms and QoL. Data were also collected for mortality, adverse effects of NIV, pulmonary function tests and polysomnography indices. Details of effectiveness data were obtained from clinical records and questionnaires completed before and after NIV. QoL was measured using the Paediatric Quality of Life Inventory (PedsQL). Parents and children evaluated QoL in several different areas, before and after NIV, using both PedsQL4.0 generic and neuromuscular modules. Different PedsQL were used according to the age of the patients and scores were converted to a 0 to 100% scale.

Effectiveness results
Nine patients were reported as having daytime somnolence and 4 as having headaches prior to NIV. Following NIV, no patient reported daytime somnolence, (p=0.003), and 1 continued to report headaches, (p=0.046).

Before NIV, 7 patients were reported as having anorexia and 4 had failure to thrive (weight below the third centile for age). After NIV, 3 patients were reported as having anorexia, but 5 had failure to thrive.

Five patients experienced adverse effects related to NIV and 1 patient died.

As the children enrolled in the study had progressive neuromuscular disorders, it would be expected that their QoL would deteriorate with increasing age. The study findings revealed that QoL remained stable after NIV despite disease progression. Seventy-nine per cent of the parents completed both QoL questionnaires before and after NIV and no significant deterioration in parent-reported QoL was found.

Three children and 4 teenagers completed child report forms after NIV only. The PedsQL4.0 generic total QoL ranged from 32 to 77.9% for children and from 32.1 to 79.1% for teenagers. Interclass correlation between the child- and adult-reported QoL was 0.94, (p=0.002).

Only 3 patients underwent pulmonary function testing at the two stages of time (before and after NIV). No change in pulmonary function was revealed.

Clinical conclusions
The use of NIV in the treatment of respiratory failure in children with neuromuscular disease resulted in a reduction in symptoms, without adverse effects on QoL.

Measure of benefits used in the economic analysis
The authors did not derive a summary measure of benefit. In effect, a cost-consequences analysis was performed. See the ‘Analysis of Effectiveness’ section for the clinical outcomes measured. The benefits were not discounted.

Direct costs
The authors included direct costs before and after the institution of NIV. The direct costs included before NIV were obtained from the finance department of the tertiary hospital, the patients’ local hospitals and the ambulance service, and were associated with outpatient visits and hospital admissions. Additional costs after NIV included equipment and specialised nursing costs. The costs were reported as the mean annual direct cost of health care per patient. Discounting was not conducted and the price year was not reported.

Statistical analysis of costs
Health care costs before and after NIV were compared using the Wilcoxon non-parametric related sample test.
Indirect Costs
Productivity costs were not relevant to the perspective adopted.

Currency
Australian dollars (AUD).

Sensitivity analysis
The authors did not address uncertainty.

Estimated benefits used in the economic analysis
See the 'Effectiveness Results' section.

Cost results
The mean annual direct health care cost per patient decreased from AUD 55,129 prior to the institution of NIV, to AUD 14,914 in the year after NIV, (p=0.003).

Synthesis of costs and benefits
No synthesis was performed.

Authors' conclusions
The treatment of respiratory failure in children with neuromuscular disease with NIV resulted in a reduction in symptoms, hospitalisations and health care costs, without adverse effects on QoL.

CRD COMMENTARY - Selection of comparators
The authors appear to have compared NIV with an invasive artificial airway. However, we cannot be certain as no information on the standard practice was provided. You should decide if the comparator represents current practice in your own setting.

Validity of estimate of measure of effectiveness
The clinical study was a within-group comparison study. The non-randomised, non-parallel design introduces considerable potential for confounding in the results. You should consider if there is any reason to believe that baseline values would not remain constant or deteriorate through the follow-up period. Furthermore, evidence came from a single centre which might not have been representative of the entire population. No power calculations were reported to have been used to determine the sample size; the small number of patients limits the robustness of the analysis. No sensitivity analysis of the results was performed.

Validity of estimate of measure of benefit
The authors did not derive a summary measure of benefit. In effect, a cost-consequences analysis was performed. You should consider if the clinical outcomes adequately represent the health outcomes relevant to the intervention.

Validity of estimate of costs
The perspective adopted in the study was not reported and for that reason it is difficult to ascertain whether all the relevant costs were included in the analysis. As the authors acknowledged, the total costs might have been underestimated as visits to general practitioners and medications obtained externally were not included. Furthermore, it appears that the costs of psychotherapy were also not included; it is not clear if this biases the results for or against the intervention. The authors did not address uncertainty surrounding the cost estimates. Although methodologically necessary, the authors did not carry out any discounting. It would have been helpful had the authors provided a more detailed breakdown of the costs in order to facilitate the understanding of the main cost-drivers.
Other issues
The authors did not compare their results with the findings from other studies. In addition, the issue of generalisability to other settings was not addressed. The authors do not appear to have reported their results selectively. As the authors acknowledged, results relating NIV with QoL must be interpreted with caution as the QoL data prior to NIV might have been affected by recall bias. The authors’ conclusions reflected the scope of their analysis, but the study was limited in the aspects mentioned above.

Implications of the study
Recommendations for further research were made for this patient group: the authors suggested that future research should address the use of pulmonary function assessment techniques that do not require patient cooperation. Furthermore, the authors recommended that future research should focus on reliable diagnosis of respiratory insufficiency and the optimal time of institution of NIV in children.

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