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Pediatric home mechanical ventilation: A Canadian Thoracic Society clinical practice guideline executive summary

Reshma Amin, MD, FRCPC, MSc^{a,b,*}, Ian MacLusky, MBBS, FRCPC^{c,*}, David Zielinski, MD FRCPC^{d,*}, Robert Adderley, MD, FRCPC^e, Franco Carnevale, RN, PhD^f, Jackie Chiang, MD, FRCPC^g, Aurore Côté, MD^d, Cathy Daniels, RN, NP-Paed^b, Patrick Daigneault, MD, FRCPC^h, Christine Harrison^o, Sherri Katz, MDCM, FRCPC, MSc^c, Krista Keilty, NP-Paed, PhD^{b,p}, Carina Majaesic, MD^j, Theo J. Moraes, MD, PhD^{a,b}, April Price, MD^k, Dhenuka Radhakrishnan, MD^c, Adam Rapoport, MD, FRCPC, MHSc^{b,l}, Sheldon Spier, MD, FRCPCⁱ, Surendran Thavagnanam, MD, MRCPCH^m, Manisha Witmans, MD, FRCPCⁿ, and Canadian Thoracic Society

^aDivision of Respiratory Medicine, The Hospital for Sick Children, Toronto, Canada; ^bUniversity of Toronto, Toronto, Canada; ^cDivision of Respiratory Medicine, Children's Hospital of Eastern Ontario, Ottawa, Canada; ^dDivision of Respiratory Medicine, Montreal Children's Hospital, Montreal, Canada; ^eDivision of Paediatric Critical Care, British Columbia Children's Hospital, Vancouver, Canada; ^fDivision of Paediatric Critical Care, Montreal's Children's Hospital, Montreal, Canada; ^gDivision of Complex Continuing Care, Holland Bloorview Kids Rehabilitation Hospital, Toronto, Canada; ^hDivision of Paediatric Respiriology, Centre Mère-Enfant Soleil du Centre Hospitalier de l'Université Laval, Québec, Canada; ⁱDivision of Paediatric Respiratory Medicine, University of Calgary, Calgary, Canada; ^jDivision Paediatric Respiratory Medicine, Stollery Children's Hospital, Edmonton, Canada; ^kDivision of Paediatric Respiriology, London Health Sciences Centre Children's Hospital, London, Canada; ^lPaediatric Advanced Care Team (PACT), The Hospital for Sick Children, Toronto, Canada; ^mDivision of Paediatric Respiratory Medicine, University Malaya Medical Centre, Kuala Lumpur, Malaysia; ⁿDepartment of Medicine and Dentistry, University of Alberta, Edmonton, Canada; ^oDepartment of Bioethics, The Hospital for Sick Children, Toronto, Canada; ^pCentre for Innovation and Excellence in Child & Family Centred-Care, The Hospital for Sick Children, Toronto, Canada

ABSTRACT

Over the last 30 to 40 years, improvements in technology, as well as changing clinical practice regarding the appropriateness of long-term ventilation in patients with “non-curable” disorders, have resulted in increasing numbers of children surviving what were previously considered fatal conditions. This has come but at the expense of requiring ongoing, long-term prolonged mechanical ventilation (both invasive and noninvasive). Although there are many publications pertaining to specific aspects of home mechanical ventilation (HMV) in children, there are few comprehensive guidelines that bring together all of the current literature. In 2011 the Canadian Thoracic Society HMV Guideline Committee published a review of the available English literature on topics related to HMV in adults, and completed a detailed guideline that will help standardize and improve the assessment and management of individuals requiring noninvasive or invasive HMV. This current document is intended to be a companion to the 2011 guidelines, concentrating on the issues that are either unique to children on HMV (individuals under 18 years of age), or where common pediatric practice diverges significantly from that employed in adults on long-term home ventilation. As with the adult guidelines,¹ this document provides a disease-specific review of illnesses associated with the necessity for long-term ventilation in children, including children with chronic lung disease, spinal muscle atrophy, muscular dystrophies, kyphoscoliosis, obesity hypoventilation syndrome, and central hypoventilation syndromes. It also covers important common themes such as airway clearance, the ethics of initiation of long-term ventilation in individuals unable to give consent, the process of transition to home and to adult centers, and the impact, both financial, as well as social, that this may have on the child's families and caregivers. The guidelines have been extensively reviewed by international experts, allied health professionals and target audiences. They will be updated on a regular basis to incorporate any new information.

KEYWORDS

Airway clearance strategies; central hypoventilation syndrome; chronic obstructive pulmonary diseases; Duchenne muscular dystrophy; ethics; home mechanical ventilation; kyphoscoliosis; muscular dystrophies; myopathies; myotonic dystrophy; obesity hypoventilation syndrome; prolonged mechanical ventilation; spinal muscle atrophy; transition

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Section 1: Introduction

Similar to that in adult medicine, improvements in available technology and clinical care have resulted in increasing

numbers of children being placed on long-term home mechanical ventilation (HMV). There has been a growing realization that long-term institutional care is not the optimal environment for these children's emotional and intellectual growth. Along with the rising costs of hospital care, and the family's appropriate desire to care for these children within their own family environment, there has been greater demand for HMV in children. The present HMV clinical practice guideline is intended to be a resource for physicians, health care providers,

policy makers and families of individuals at risk for or currently using ventilatory support in the home. The objective is to identify and support ventilated children who are presently at home, as well as those transitioning to home-based care where the quality of life (QoL) is greatest and costs are minimized. Developed by the Canadian Thoracic Society (CTS), these guidelines intend to provide the most up-to-date information and evidence-based recommendations to enable practitioners to manage the provision of preventive airway management and HMV.

These guidelines are composed of disease-specific sections in addition to overriding subjects such as ethical considerations, transition to home, transition to adult care, and airway clearance. Within this sphere of practice, even within adult medicine, there are extremely few prospective or randomized trials, and even fewer in children.

Again, as with the adult guidelines, the recommendations provided strive to achieve a balance between an exceptional standard of care illustrated in the literature and the reality of health care in Canada, where geographical and economic barriers may require adaptation, to ensure feasible yet safe care. This approach may also allow greater applicability to jurisdictions where, for example, polysomnography (PSG) may be unavailable or so difficult to obtain due to present barriers to appropriate, timely introduction and/or follow-up of noninvasive ventilation (NIV). As with the adult guidelines, some subjects considered to be important by the committee are not addressed in the literature. On important issues for which literature is lacking, but strong expert opinion was available, recommendations were made by the HMV Guideline Committee.

Preventive airway management and HMV in children is a complex, interdisciplinary component of respiratory care and expert clinical practice. There is a continuum of chronic disease management involving many layers of expertise from government and professional education to home care services, acute and chronic health facilities and independent living facilities.

The present document represents the executive summary of the guideline. The source document, which is more detailed and includes research questions identified by the committee, and evidence tables, is available online at www.respiratoryguidelines.ca/guideline/home-ventilation-children.

Question

What evidence is available to inform the practice of HMV in children and lead to better individual, caregiver, and system outcomes?

Objective

The objective of the present clinical practice guideline is to provide guidance on the role of mechanical ventilation for children in the home setting. A guideline on this topic is needed to inform best practices, provide a basis to identify gaps in care and provide direction for future research. Specifically this guideline focuses on invasive ventilation and positive pressure noninvasive ventilation (NIV). Continuous

positive pressure ventilation (CPAP) is not discussed. Additionally, negative pressure and positive pressure body ventilators have been excluded from these guidelines given that they have been replaced with portable positive pressure ventilators.

Target population

The current clinical practice guideline applies to all children who are at risk for or are using HMV. Individuals with spinal muscular atrophy (SMA), central hypoventilation syndrome, specifically congenital (CCHS), chronic lung disease of infancy (CLDI), kyphoscoliosis, obesity hypoventilation syndrome (OHS), Duchenne muscular dystrophy (DMD), and muscular dystrophies (MDs) other than DMD, are of special interest and are considered individually in the present clinical practice guideline.

Target users

The present clinical practice guideline is intended for use by the health care teams that care for individuals who are at risk for or require ventilatory assistance. Respiriologists, physiatrists, neurologists, family practitioners and community pediatricians, nurses, respiratory therapists, physiotherapists, and other health care professionals can use these guidelines to help inform their clinical practice with regard to HMV.

Methodology

Guideline development

This clinical practice guideline was developed according to the convention of the 23-item AGREE II instrument – the current gold standard in the appraisal of practice guidelines.¹ The HMV Expert Committee was interprofessional comprised of HMV clinicians with content expertise in each of the topic areas. An initial systematic review of the literature was completed current to June 2010. A second review of the literature was also performed through to August 2015. In accordance with the CTS guideline review process, before completion, CTS staff distributed the guideline for formal review by: 1) 3 international content experts, and 2) 2 national reviewers that rated the guideline using the AGREE II instrument. These reviews and AGREE II checklists were provided to the CTS guidelines committee. We then provided responses to the comments and made corresponding changes to the document. The authors were blinded to the identities of the reviewers. All reviews and author responses are posted, along with author conflicts of interest, at respiratoryguidelines.ca.

Final consensus on the recommendations from the CTS HMV Committee was reached through a formal voting process that was anonymized. In accordance with the Canadian Thoracic Society's Guideline update process, these guidelines will be reviewed every 3 years or sooner.

Table 1. Grading recommendations.

Grade of recommendation/ description	Benefit versus risk and burdens	Methodological quality of supporting evidence	Implications
1A/strong recommendation, high-quality evidence	Benefits clearly outweigh risk and burdens, or vice versa	RCTs* without important limitations or overwhelming evidence from observational studies	Strong recommendation, can apply to most patients in most circumstances without reservation
1B/strong recommendation, moderate quality evidence	Benefits clearly outweigh risk and burdens, or vice versa	RCTs with important limitations (inconsistent results, methodological flaws, indirect or imprecise) or exceptionally strong evidence from observational studies	Strong recommendation, can apply to most patients in most circumstances without reservation
1C/strong recommendation, low-quality or very low-quality evidence	Benefits clearly outweigh risk and burdens, or vice versa	Observational studies or case series	Strong recommendation but may change when higher quality evidence becomes available
2A/weak recommendation, high-quality evidence	Benefits closely balanced with risks and burden	RCTs without important limitations or overwhelming evidence from observational studies	Weak recommendation, best action may differ depending on circumstances, patients' or social values
2B/weak recommendation, moderate-quality evidence	Benefits closely balanced with risks and burden	RCTs with important limitations (inconsistent results, methodological flaws, indirect or imprecise) or exceptionally strong evidence from observational studies	Weak recommendation, best action may differ depending on circumstances, patients' or social values
2C/weak recommendation, low-quality or very low-quality evidence	Uncertainty in the estimates of benefits, risks and burden; benefits, risk and burden may be closely balanced	Observational studies or case series	Very weak recommendations; other alternatives may be equally reasonable

Note. *RCTs Randomized controlled trials.

Literature search strategy

An initial literature was searched using MEDLINE (OVID: 1980 through June 2010), Embase (OVID: 1980 through June 2010), HealthStar (1980 through June 2010), the Cochrane Library (OVID: Issue 1, 2009), the Canadian Medical Association InfoBase, and the National Guideline Clearinghouse. Reference lists of related papers and recent review articles were also scanned for additional citations. The literature search was repeated in 2015 (through to August 24, 2015), in order to include the most recent literature. Major relevant guidelines published during the review period in 2016 were also incorporated into the document.³

Study selection criteria

Articles were selected for inclusion in the systematic review of the evidence if they reported data on the role of HMV in children.

Critical appraisal

The strengths and weaknesses of the evidence were carefully considered in the generation of the recommendations. Although the majority of the evidence in this topic area is modest, the Grading of Recommendations Assessment, Development and Evaluation (GRADE) methodology was used to inform the generation of recommendations and critically appraise the strength of the evidence.² When no evidence was available, the committee made a recommendation when consensus was reached; the recommendation was subsequently identified as such (Table 1).

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Section 2: Initiation of long-term mechanical ventilation and transition to home

Introduction

Despite its widespread use there remains very little comparative literature on the precise indications and patient selection process, timing, or optimal methodology for successful initiation of HMV. This is reflected in the marked variability in frequency, types of patients and patterns of HMV reported between different centers, while conditions deemed appropriate for HMV continue to evolve. What literature there is generally describes which individuals were placed on long-term ventilation (LTV) at home, how ventilation was initiated, and some outcome data (the majority of this data including all ages, with relatively little pediatric-specific data). Most programs have developed their own criteria and protocols for initiation of home ventilation based largely on empiric experience. Although these protocols are generally fairly similar, being based on similar experience and therapeutic goals, there is still a lack of agreement, and even less objective data, as to precisely

who would benefit from long-term HMV. These protocols generally outline the training that is needed, and the equipment needed for the safe transition of the child to the home. Driven by costs, recognition that long-term institutionalization is a far from ideal environment for both child and family, and development of technology that allows for the children on long-term ventilation to be safely cared for at home, in almost every program the stated goal is for LTV to occur within the home environment. Given that many of these children, by definition, are facing life-limiting illnesses, and that caring for these children at home has a huge impact on family finances, and family functioning, the relative absence of objective evidence providing support for these protocols is a significant deficit in our knowledge.

Key evidence

Initiation of ventilation

1. Indications for initiation of HMV: Several criteria have been suggested as indications for initiation of HMV.

a. Prolongation of life.

i. Noninvasive ventilation (NIV). The benefit of noninvasive ventilation has been well established in specific populations through cohort studies showing increased survival compared to the natural history of the disease without support.¹⁻⁴ To date there have been 2 RCTs performed looking at this. One study that evaluated “prophylactic” initiation of NIV was terminated early because of a 4-fold increase in mortality rate in patients on NIV.⁵ However, there have been significant methodological issues raised with this study and it is not clear whether these patients had already developed sleep disordered breathing at the time of initiation. A second smaller RCT in children with various neuromuscular or chest wall diseases and nocturnal hypoventilation, randomized to either NIV or spontaneous breathing at night showed no change in mortality. However 9 of 10 controls required institution of ventilatory support within an average of 8.3 months. In addition, patients with nocturnal hypoventilation will progress to daytime hypercapnia within 12–24 months.⁵ Given the significant established increase in prolongation of life in cohort studies, further RCT looking at this question would not be ethical.

ii. Invasive ventilation. For a child in end-stage respiratory failure in whom noninvasive ventilation, is not an option, invasive ventilation remains the only option for long-term survival. Reasons, NIV may not be an option include: 1) ventilator support is required for > 16 hours a day and the child is not a candidate for mouth-piece ventilation; 2) ventilation cannot be managed with NIV; oral secretions or uncontrollable gastroesophageal reflux disease prohibit the safe use of NIV; a mask interface cannot be tolerated.

The decision to proceed with invasive ventilation via tracheostomy involves a discussion surrounding the individual’s expected outcome, and QoL. This obviously precludes the performance of randomized control trials.

b. Quality of Life.

i. The effect of HMV on QoL is discussed in Section 3 Ethics and in the disease specific sections.

c. Prevent Deterioration.

i. Prevention of either deterioration in pulmonary function or respiratory exacerbations. Several studies have shown that initiation of NIV fails to prevent subsequent deterioration in pulmonary function.^{6,7} NIV does, however, reduce the probability of requiring readmission, at least within the year following its initiation.⁷

2. Identifying the long-term home mechanical ventilation (HMV) candidate: Children who are being considered for HMV have life-limiting conditions. There is, however, very little documentation as to the process to be used to identify the individual patient’s, as well as their families’, suitability for placement on HMV. HMV may have a huge impact on not only the child’s QoL and life expectancy, but also the entire family’s QoL, free time, and financial well-being.⁸⁻¹⁰ This is further compounded by the marked variability in resource availability, not only within the community but also between families. Protocols are needed that address all of these issues prior to initiation of ventilation, with initiation of ventilation occurring as part of a structured plan.

a. Screening. There is good evidence that in children with progressive neuromotor or pulmonary disease there is a predictable evolution in their respiratory failure.¹¹ It is also well recognized that this can be an insidious process (obviously being altered or hastened by respiratory infection or other impairments of respiratory function). Patient symptoms and clinical evaluation may be insufficient for reliable diagnosis.¹² Therefore, objective measures including PSG, oximetry and capnography are required. See Section 8 for more details.

b. Location for initiation. Initiation of ventilation can occur in hospital (ward or intensive care unit (ICU), in the sleep laboratory, or even as an outpatient, being dependent not only on the child’s clinical status, but also on the local resources, particularly the availability of knowledgeable personnel, and the ability to perform comprehensive monitoring to assess adequacy of ventilation. Family preferences and capacity for decision making also need to be evaluated. The one randomized trial on NIV did show that it could be initiated just as successfully in an outpatient as in an inpatient setting.¹³ It is therefore predicated primarily by local resources including access to PSG or oximetry/capnography in the community. Of note, the initiation of invasive ventilation will always occur in an ICU setting.

- c. Ability to care for the child in the home environment. The ability of the family to care for the child needs to be carefully evaluated. The child should be medically stable (unless palliative), including the presence of a stable airway, stable oxygen requirements (usually less than 40%), blood CO₂ must be maintained within safe limits on the home ventilator, and nutritional intake must be adequate to maintain expected growth and development. All other health conditions, pain and dyspnea included, should be well controlled. It also must be feasible to provide the level of support and intervention that the child will need at home. There should be no need for laboratory tests or changes in treatments to ensure community caregivers can manage the child in the home environment. There should also be no changes to the medical plan. The parents need to understand the long term prognosis and must be willing and capable to meet the child's complex needs in the home environment.¹⁴ See Section 3 Ethics for further details.
3. Preparation of family and patient: Families with a child who has chronic respiratory failure must choose between accepting long-term assisted ventilation and forgoing life-assisting technology for their child.¹³
- a. The patient and their family need to have a clear understanding of the potential benefits as well as the risks for HMV in their child, either noninvasive or invasive. Most published protocols emphasize the need for the patients and their families to be given adequate time and information prior to initiation of HMV to ensure appropriate planning and ability to facilitate informed consent. The discussion regarding the possibility of HMV should therefore commence close to the time of initial diagnosis of the illness, be progressively discussed as the disease advances, and certainly mentioned long before imminent respiratory failure.¹⁵ Unfortunately, even when the child's disease has a predictable course and outcome, all too often this does not occur.¹⁵
- b. The medical team as well as the patient and family need to all be in agreement with the decision to move forward with HMV.

Conclusions

HMV has become an accepted therapy for a variety of diseases. Although multiple protocols exist, these are largely based on clinical experience, with very little comparative data to evaluate the optimal methodology and selection criteria. Given the reported prolonged length of stay as well as the barriers that are reported to delay discharge, we have outlined below the key components of the process for initiating and discharging a child on LTV. These considerations are essential to facilitate a timely and safe transition from hospital to home (see Appendix 1 in the source documents available at www.respiratoryguidelines.ca/guideline/home-ventilation-children).

Recommendations for initiation of long-term mechanical ventilation at home

The following recommendations are based on limited evidence from studies and the use of expert consensus of the HMV expert panel.

Initiation of long-term ventilation in children

1. *Each institution caring for children at risk of respiratory failure should establish a screening protocol (suggestions: symptoms review, routine polysomnogram [oximetry and capnography if polysomnogram is not available, pulmonary function]), for the identification of sleep disordered breathing. (GRADE 1C)*
2. *Each institution caring for children at risk of respiratory failure should establish protocols for the initiation of HMV, with initiation of ventilation occurring as part of a structured plan of care. (GRADE 1C)*
3. *Wherever possible the patients and their families should be given sufficient time and information to allow them to make an informed decision regarding advanced care planning prior to initiation of ventilation. Initial discussions as to the possibility of HMV should begin at the time of diagnosis and revisited progressively as the disease progresses and according to the needs of the family. (GRADE 1C)*
4. *A national registry is needed to track which patients are initiated on HMV, the indications and technologies employed, as well as patient outcomes, to provide objective data on the exact role and indications for HMV in individual patients. (Consensus)*

Transition to home

Although NIV can often be started outside of a hospital setting, invasive ventilation is done as an inpatient. The reported range for length of stay after initiation of invasive ventilation was wide and ranged between 46 days to 9.6 months.^{16–23} Barriers that are encountered during the discharge process can be classified into eight categories: 1) housing issues (eg, lack of running water or electricity); 2) funding of equipment; 3) receiving equipment for the home; 4) social issues related to the family; 5) intercurrent illness of the child; 6) issues with community caregivers (eg, inconsistent caregivers, lack of training of caregivers in the community) or arranging out of home placements; 7) lack of a coordinated approach to discharge and the absence of a clear coordinator; and 8) delays in organizational decision making and attitudes of professionals.^{17–19,21,24–25} For example, some parental caregivers have reported that their children stayed in hospital for prolonged periods of time because professionals did not think the families could cope at home and others felt that they were safer in hospital.

Ideally, protocols should exist that address these issues prior to initiation of ventilation, with initiation of ventilation occurring as part of a structured plan, thereby increasing the probability of long-term success. Once the decision has been made to proceed with HMV, discharge planning should begin immediately. The course in hospital can be subdivided into three main categories: i) establishing the mechanical ventilation

parameters for the transition to home; ii) caregiver training; iii) discharge planning.

1. Ventilation parameters for the transition to home: As soon as the patient is deemed medically stable and the decision has been made to go ahead with domiciliary HMV, work should begin to determine the appropriate ventilator model, mode of ventilation and settings. The patient should be trialed on potential home ventilators to ensure that their gas exchange can be adequately managed on an appropriate home unit. An early introduction of the home ventilator will also facilitate adherence and serve to identify any pitfalls early. For NIV, including mask ventilation or mouthpiece ventilation, the interfaces also need to be trialed to ensure comfort and effectiveness. For mask NIV, interface leak must also be assessed and minimized. Oximetry, transcutaneous CO₂ measurements, blood gases and bedside assessments can be used to titrate the settings and maximize the time off the ventilator in a 24-hour period. Where appropriate for children receiving invasive HMV, readiness for a speaking valve should also be investigated. In general, it is recommended that children are on stable ventilator settings with adequate gas exchange for ≥ 2 weeks prior to discharge home for invasive HMV and ideally for 48 hours for NIV depending on the fragility of the child.
2. Caregiver training requirements: Caregiver training is dependent upon several factors.
 - a. Medical fragility of the child. There is an interrelationship between the medical fragility of the child, their ability to self-care, and the complexity of the care they require. This is influenced by several factors which include the following:
 - i. Children who are dependent on ventilation for survival (failure of ventilatory support will lead to rapid demise) are more fragile than those who are able to breathe without assistance on their own (eg, those with neuromotor disabilities).
 - ii. Many children may also have other impairments that require additional technological support, such as enterostomy feeds, catheterization, etc.
 - iii. The child can verbalize and is able to instruct the caregiver on needs.
 - b. Mode of ventilation (ie, invasive via tracheostomy or NIV) and hence the complexity of equipment required has a significant impact on the depth of caregiver training required, the most intense being for children on invasive ventilation. These patients require caregivers who are knowledgeable not only in ventilator but also tracheostomy management, with tracheostomy-related events being a principal cause of death in these patients.²⁶ Since adverse events can occur at any time it is generally recommended that a child with a tracheostomy have “24/7” care by an awake, alert, trained caregiver.^{27–29} This requires a minimum of 2 caregivers identified who are able and willing to be trained in tracheostomy and ventilator care in order to care for the child at home. The length of the caregiver training program varies, usually in the order of 6 to 8 weeks (for invasive HMV and less

than 1 week for NIV), but depends on the readiness, availability, and abilities of the designated caregivers for training.^{27–28} The family should also receive support to care for the child in the community by paid caregivers. However, the number and type of paid community caregiver hours available to families varies across regions and provinces. Many families are therefore forced to rely on other supports, such as unpaid caregivers, family members, nannies, etc. The issue facing these families, and the child’s health care providers, is to ensure that these individuals have sufficient knowledge and education to safely care for their children. A formal training schedule organized by the HMV should therefore be in place for all of the child’s caregivers. Although most programs have similar protocols, there are no validated criteria outlining the needed knowledge and skills necessary to care for a child on HMV. Most programs emphasize the need for a period of care-by-parent as the last step prior to discharge, when the child remains an inpatient, where help is nearby if needed, while the family caregivers are responsible for the ongoing care of the child. In addition, all family caregivers must be Basic Life Support (BLS) certified prior to the child’s discharge home. Close to the time of discharge, the family caregivers should have their knowledge and skills reassessed.

- c. Ability of the child to self-care. Obviously infants and young children are dependent upon continuous provision of care by adult caregivers. The long-term goal, however, is for the child to achieve as much independence as their illness and developmental level allows. Consequently the child should also participate, within the limits of their abilities, in the training program and be encouraged to assume as much of a role in their self-care as they wish and are capable of.
3. Financial considerations: Given the necessity of “24/7 eyes on care by an awake, alert, trained caregiver,”²⁹ one parent often has to stop working to become the primary caregiver. This results in a significant income loss for the family. Although most local governments provide some financial support for the required equipment, the relative contribution to the cost of the ventilator and needed supplies varies across provinces, and is rarely sufficient to cover all expenses. Furthermore, transportation costs (eg, wheelchair-accessible vans), costs associated with mobility devices (eg, wheelchairs) as well as the cost of extra supplies during illness are all added expenditures for the family. This inevitably results in a significant financial burden to most families.
4. Housing assessment: All children going home on HMV require a housing assessment to determine suitability, which includes adequate space for equipment and supplies, including doorways (wheelchair), adaptations for home oxygen, adequate electrical outlets, telephone access in case of emergency, functional heating and cooling systems, etc. Ideally the ventilator-dependent child would have their own room with adequate storage for supplies and equipment. An evacuation process in case

of fire should be established. Priority for re-connection of electricity in the event of power loss should also be instigated.

5. Community services: The links to the community care services (eg, home care nursing, rehabilitation services) should be established well in advance of the discharge date. The community-based homecare providers should be identified early to ensure both that there is adequate coverage, and that the community caregivers receive adequate training. Potentially relevant factors include: degree of ventilator dependence, medical fragility (see 2a, above) (will the child awaken or breathe spontaneously if disconnected, use of invasive HMV or NIV), mode of nutrition, ambulatory or wheelchair assisted, other health issues, as well as the ability of the family to cope with the child's needs.¹⁴ Therefore, it is essential that community partners are identified as early as possible in the process to prevent discharge delays.
6. Discharge planning: A multidisciplinary assessment including the family, the patient, intensive care, respiratory medicine, nursing, social work, occupational therapy, physical therapy, respiratory therapy, the primary care pediatric provider, the community homecare agency and discharge planning should be performed to identify the particular needs of the child and family. The inter-professional teams need to be coordinated by one primary case manager. The benefits of a dedicated case coordinator for a patient have been demonstrated.²² The case manager needs to act as a liaison between the hospital-based teams and the community care providers. Some discharge programs will have 2 coordinators—a community practitioner and a hospital case manager, or vice versa.^{14,30} As soon as a decision has been made to proceed with HMV at home regular multidisciplinary team meetings should be scheduled every 4 weeks and a projected discharge date should be set.

For discharge from hospital to home

1. Discharge care plan: A written discharge care plan is an essential component of a successful discharge from hospital to home. This should include the clinical information around ventilator dependence, the underlying disease and prognosis, the current ventilator settings, a plan in the case of acute illness or deterioration, what to do in case of emergencies, as well as the names and contact information of the main health care advisors, as well as emergency services. This treatment plan should be shared with the family, as well as all necessary local agencies, including ambulance, police, and local utilities. The health care team must ensure the family has an adequate mode of transportation to transport the child to follow-up appointments as well as around the community, with an additional caregiver in the vehicle for children who require ventilation during transit, or have a tracheostomy. If feasible, a graduated discharge with a 'trial run' at home is recommended prior to discharge.

2. Ongoing care: Ongoing follow up and coordinated care between the local and specialized clinical teams is essential to successfully support the child and their family.²⁹ Ongoing care is provided by a joint partnership between the primary care provider and the respirologist and HMV team. This ongoing care is multimodal and may include any combination of the following: telephone, telemedicine, home visits, clinic visits, and visits during hospitalizations for intercurrent illnesses. The family therefore needs to be aware of the follow-up care plan, which will depend on the child's status and needs, and coordinated with other consultants following the child. Furthermore, family caregivers need to continue to receive ongoing tracheostomy and ventilator skills assessments and retraining.
3. School: Many children with a tracheostomy and invasive HMV attend school. An emergency plan needs to be clearly delineated for the child and a mobility plan for the child around the school needs to be clarified. In addition, a tracheostomy and ventilation-trained caregiver needs to accompany the child at school and the other allied health care services the child will receive at school need to be arranged.
4. Equipment: There is an extensive list of equipment and supplies needed to care for a ventilated child, both at home and for travel that should be developed and confirmed by the team. In addition, the source of the equipment as well as the ordering responsibility and necessary funding needs to be identified (see Section 2, Full Article, Appendix 2). Equipment and supplies should be ordered as early as possible in the transition process to avoid delays in discharge.
5. Psychosocial considerations: See Section 13 for psychosocial considerations of HMV.

Conclusions

There has been a significant increase in the number of medically complex children requiring long-term ventilation in the past few decades. The overwhelming majority of children receiving HMV are successfully discharged from the hospital to home. The success of home ventilation programs is in large part due to the coordinated, comprehensive multidisciplinary approach driven by a central, hospital-based case manager and includes dedication and commitment by family and community caregivers.

Recommendations for transition to home

The following recommendations are based on limited evidence from 10 studies and Consensus of the HMV expert panel.

Transition of children on long-term ventilation to home

1. *Each institution caring for children on long-term mechanical ventilation at home should collaboratively develop a written care plan, with the family, health care providers in the referring center, as well as the community health care/homecare team involved in the patient's long-term care. (GRADE 1C)*

2. **Lines of communication need to be established between regional centers and the community caregivers, with clear outlines as to respective responsibilities both for routine follow up, and emergencies associated with unexpected events such as illness or equipment failure. (GRADE 1C)**

The following recommendations have been adapted from the adult home mechanical ventilation guidelines and are based on the consensus of the expert pediatric HMV panel.

3. **Each institution discharging a child home should have a designated case coordinator to streamline the discharge process, minimize the length of stay, and ensure a safe transition home. (Consensus)**
4. **The patient should be medically stable with a need for a level of monitoring or treatment interventions that can be managed at home. (Consensus)**
5. **The patient and the family must be highly motivated. (Consensus)**
 - a. **The family is willing/able to ensure provision of “24/7 eyes on care” for all invasively ventilated children and for children receiving NIV that are deemed medically fragile and in need of this level of care at home.**
 - b. **The family is aware that the discharge destination is home, not a long-term care facility.**
 - c. **The family is willing to provide needed supports (financial, physical, emotional).**
6. **The patient must have a home that is safe for a child requiring long-term mechanical ventilation. (Consensus)**
7. **The patient must have sufficient caregiver support. (Consensus)**
 - a. **Two primary, family caregivers identified and trained.**
 - b. **Sufficient community caregivers identified and trained to provide parental support. In the absence of private funding, sufficient government funding available to pay for these caregivers.**
 - c. **Sufficient out-of-home respite needs to be available.**
 - d. **Family caregivers in agreement that they are responsible for the majority of the care.**
8. **Each institution or HMV program caring for these children must ensure the patient has the appropriate equipment and supplies needed for long-term ventilation. (Consensus)**
 - a. **There must be a list of needed equipment and the institution or HMV program must ensure that it is ordered and delivered to the home or is in use.**
 - b. **Sources for ongoing required supplies need to have been identified.**
 - c. **There should be a clear protocol in place for maintenance and replacement of necessary equipment.**
9. **There must be comprehensive initial competency-based training regarding the knowledge and skills needed to care for a child using HMV, plus ongoing education and training for patient and caregivers once they are in the home setting.²⁹ (Consensus)**
 - a. **The initial education is organized to accommodate learning, practice, and inclusion of caregivers in the care routine as early as possible.**

10. **The patient must have access to health care support in the community. (Consensus)**

- a. **Follow-up care is available as appropriate (tracheotomy tube changes, ventilator reassessments, interface fit and skin breakdown, adherence and assessment of the ongoing effectiveness of the ventilator support).**
- b. **Medical follow-up to allow for appropriate changes to the mode of ventilation (ie, from invasive to non-invasive and vice versa, from continuous to nocturnal and vice versa).**

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Section 3: Ethics of initiation of long-term mechanical ventilation at home in children

Introduction

The decision to initiate long-term ventilation in children is usually based on the conclusion that it is required to mitigate the underlying disease; prolonging a child's life and sustaining quality of life (QoL) are chief among the goals of instituting this technology. Indeed commencing long-term ventilation when the option is deemed medically appropriate typically results in prolonged survival, whether invasive (eg, tracheostomy) or noninvasive (NIV) techniques are employed.^{1–3} Nonetheless, health care practitioners, patients, and families must consider more than just “will this intervention prolong life?” before a decision to initiate long-term ventilation is reached. An approach to ethical decision making regarding initiation of long-term ventilation is critical.

Key evidence

A review of the medical literature on the ethical issues associated with long-term ventilation for children at home revealed a paucity of data. The evidence suggests that a shared decision making approach is preferred by most families faced with the decision of long-term mechanical ventilation in their children.^{1,4} Shared decision making occurs when health care practitioners assist families by exploring goals of care and making

recommendations in line with those goals in order to reach a consensual decision. As with any medical decision for children who lack capacity, the child's best interests should guide decision making; however the incapable child should be involved to the extent that he or she is able and willing to participate, with their wishes being an important consideration.^{5,6} In contrast, consent must be obtained from the child who is deemed to have the capacity to make such a decision on their own. Early advanced care planning discussions with families when the possibility of long-term mechanical ventilation is foreseeable encourages them to think about their wishes prior to the time a decision is required, ideally removed from a crisis.⁷ The diagnosis of a life-limiting condition or the decision to pursue an approach focused on comfort does not prohibit the use of mechanical ventilation at home; these interventions have not only been shown to help some children live longer, they may also help them to feel better and engage in meaningful activities.^{8–11} Mechanical ventilation can also be used to help transfer children home for end-of-life care, where it can then be compassionately removed leading to a peaceful death.^{12,13}

Conclusion

Numerous ethical issues are associated with the consideration of implementing long-term mechanical ventilation at home in children. Despite the relative deficiency of medical literature on this topic to guide an evidence-based approach, long-standing ethical norms and standards to decision making for children should be used by patients, families and members of the health care team as they contemplate embarking on the use of mechanical ventilation at home. The use of this technology depends largely on the goals of care expressed by the patient and/or family. HMV may be used within the context of goals focused on palliation and comfort. Clinicians have a duty to help decision-makers appreciate: the ability of mechanical ventilation at home to achieve their goals; the challenges, financial and otherwise, that come with this intervention; and a clear picture of what life may look like once this path is taken. Lastly, this chapter explores the potential application for mechanical ventilation in children at home within the context of goals focused on palliation and comfort.

Recommendations for ethics of long-term mechanical ventilation at home in children

1. *The initiation of long-term ventilation in a child should be made using a shared decision-making model, in which health care practitioners assist children and their families by reaching a consensual decision together, after the health care team has informed them about all options available under the child's specific circumstances. (Consensus)*
2. *Whenever possible, the child should be involved in all aspects of decision making, in a developmentally appropriate way, to the extent that he or she desires and to the extent possible. When the child is deemed capable of making the decision to initiate long-term ventilation, ultimately his/her consent is required. When a child lacks capacity to decide about the initiation of long-*

term ventilation, parents (or other legal guardians) must do so by considering the child's best interests and perceived QoL within the framework of family-centered care. (Consensus)

3. *The health care team should inform the child and family about all options available under the circumstances, and support them to reach a decision that is consistent with their goals and preferences. (Consensus)*
4. *Health care providers should clearly explain regional realities about resource limitations and convey how such constraints might impact the child, the parents and the family when facing a decision regarding HMV. Health care providers should not prohibit the initiation of long-term ventilation based solely on resource limitations; the best interests of the child must remain at the center of the health care team's consideration. (Consensus)*
5. *When the best interests of the child are not clear, the health care team should act in accordance with the wishes of the family. However, health care providers need not acquiesce to home ventilation when it will not achieve realistic or reasonable goals of care or when it is outside of the standard of care. (Consensus)*
6. *Advanced care planning should begin early for children in whom long-term mechanical ventilation at home is foreseeable. (Consensus)*
7. *Mechanical ventilation may have a role within the context of a palliative plan of care. It has been successfully used to help some children with life-limiting conditions be more comfortable and achieve meaningful goals. With adequate planning and resources, mechanical ventilation may also be used to transport a child in order to facilitate a compassionate death at home. (Consensus)*

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Section 4: Home monitoring and follow-up of long-term mechanical ventilation at home in children

Home monitoring

Introduction

Children on HMV are at risk of acute deteriorations and adverse events. There is an increasing number of infants and children now cared for with mechanical ventilation in the home environment.^{1–8} Hence there is a need for monitoring and close follow-up is required, especially early after discharge home. An awake and alert trained caregiver 24 hours a day and 7 days a week will provide the first line monitoring of a child receiving ventilation in a situation where interruption of ventilation is anticipated to lead to an adverse event including all invasively ventilated children as well as those on NIV that are medically fragile (at risk of imminent adverse events if the NIV mask is displaced or equipment malfunctions).⁹ Although caregivers represent the first line of monitoring for the child to identify acute events that could be potentially life-threatening, monitoring equipment is often used in addition to the built-in alarms on the home ventilators.

Key evidence

Retrospective reviews of cohorts usually from a single institution, some based on large, others on small numbers of patients, comprise most of the evidence for the risk of death and adverse outcome for children using HMV. In the absence of high-quality evidence, the strength of the recommendations were determined by consensus within the group.

Evidence comes from 30 cohort studies.^{1–31} There is ample evidence that indicates that infants on HMV are at risk of sudden death due to bleeding, infection, decannulation or disconnection from the ventilator. The evidence comes from 30 cohort studies with a total mortality of 2.4%. Thus, there is the indirect evidence that adequate monitoring should be done.

While it is counter-intuitive one might find it surprising that accidental decannulation or ventilator disconnection is frequently not detected by the low-pressure alarm on the ventilator. It has been reported that during disconnection from the ventilator, the ventilator hub might be sufficiently obstructed by the patient or by bedding material to allow creation of enough back pressure to not trigger the alarms.¹⁰ In addition, when a child has an accidental decannulation, depending on the ventilator settings and characteristics (pressure and flow), a sufficient drop in pressure may not occur. This is more likely with smaller tracheotomy tubes because of the higher resistance. One study simulated tracheotomy tube decannulation in an experimental setting with tracheotomy tubes ranging from 3.0 to 6.0 mm of internal diameter (ID).¹¹ The low inspiratory

pressure alarm was not triggered during simulated decannulation with small tracheotomy tubes (<5.0 mm ID). These findings are the basis for the recommendation of always using external monitors as well as setting the low minute ventilation alarm in addition to using external monitors. The consensus is also that patients not able to awaken or breathe spontaneously if disconnected from the ventilator require direct monitoring by a trained caregiver and should also be considered for external home monitoring.

There are several published large series of children using HMV from around the world reporting on the use of external monitors for this population, but details are scarce. In some of these programs, including Munich, Germany,¹² the Children's Hospital of Los Angeles, USA,¹³ and the Hospital for Sick Children in Toronto, Canada,⁷ all patients on invasive mechanical ventilation use pulse oximeters at home. For other programs,^{2,14} although the proportion of children under monitoring is also at or near 100%, other means of monitoring are used (eg, cardiorespiratory monitors, end-tidal CO₂ monitors, continuous direct observation by a nurse), although much less often than pulse oximeters.

Monitoring of oxygenation status is, in theory, better than that of electrocardiogram (ECG) and thoracic movement because obstructive events will not be detected with monitors that use variations in thoracic movement for respiration detection, and significant hypoxemia will precede bradycardia.¹⁴ This is the basis for the recommendation against the use of home cardio-respiratory monitors in which respiration is detected with the transthoracic impedance technique. The old generation of oximeters was, however, renowned for false alarms¹⁶; the new generation of oximeters trigger fewer false alarms and are well suited for the home environment.¹⁷⁻¹⁹ However, no evidence except consensus is available for the use of oximeters to monitor children using HMV. In addition, all monitoring equipment as well as the ventilators need to undergo regular maintenance.³²⁻³³

Conclusions

Most large studies of children using HMV report mortality due to accidental decannulation, ventilator disconnection or tracheostomy-related deaths. Home surveillance by health professionals in addition to the use of external monitors are strategies to decrease mortality and morbidity not due to progression of the underlying condition.

Recommendations for home monitoring in children using long-term mechanical ventilation at home

1. **An awake and alert trained caregiver 24 hours a day and 7 days a week will provide the first-line monitoring of a child receiving ventilation in a situation where interruption of ventilation is anticipated to lead to an adverse event including all invasively ventilated children as well as those on NIV that are medically fragile (at risk of imminent adverse events if the NIV mask is displaced or equipment malfunctions). (Consensus)**
2. **The built-in alarms of the home ventilators should be adjusted for each patient to maximize safety and minimize nuisance alarms. (Consensus)**
3. **The low minute ventilation alarm and disconnect alarm should be set optimally to maximize the detection of circuit disconnection and decannulation. (Consensus)**
4. **For children receiving invasive ventilation via tracheostomy at home through a tracheotomy tube that is smaller than 5 mm ID, an external monitor should be used in view of the potential failure of internal monitors to detect circuit disconnection and decannulation in addition to an awake and alert trained caregiver. (Grade 1C)**
5. **Patients not able to awaken, breathe spontaneously and/or at risk of adverse events if disconnected from the ventilator should have an external home monitor in addition to an awake and alert trained caregiver. (Consensus)**
6. **Home cardio-respiratory monitoring with respiration detected with transthoracic impedance techniques should not be used as external monitors as they fail to detect obstructive events. Similarly, caregivers should not rely on the detection of bradycardia with these monitors because bradycardia is a very late event. (Grade 1C)**
7. **Pulse oximeters with downloadable memory should be the monitor of choice for patients identified as needing an external home monitor. (Consensus)**
8. **Recurrent alarms should be dealt with promptly to correct the problem, whether equipment-related or patient-related. (Consensus)**
9. **Routine periodic maintenance of the home ventilators and monitoring equipment is recommended as per the manufacturer's recommendations.**

Follow-up care

Introduction

Follow-up care involves the ongoing reevaluation for the child on HMV, which includes an assessment of clinical status, the effectiveness of ventilation, and the maintenance and evaluation of the equipment. This can be done using a combination of clinic visits, in-home evaluation and telemedicine.

The frequency of follow-up visits for children on HMV is variable. It will depend on several factors including: the baseline disease and its severity, the stability of the child, the expected natural course of the disease process, the distance between home and the follow-up facility, the availability of follow-up care in the community (interprofessional team), the distance to a specialty center, and the support the parents are receiving for the care of their child (nursing support in the home). Respiratory requirements are dynamic in children due to growth, level of activity (depending on the disease) and the natural history of the underlying condition. Clinical information from visits does not always predict whether a change in respiratory support will be required and reevaluation of the support is needed on a regular basis.

Key evidence

In the few published statements from Health Authorities and Clinical Practice Guidelines,²⁰⁻²¹ we found no clear evidence-based recommendations concerning the frequency and content of follow-up visits, the use of clinic, home or telemedicine visits,

or the evaluation of ventilation effectiveness for children using HMV. Therefore, any recommendations will be based on consensus among experts. Recent randomized trials comparing clinic and telemedicine visits are available for the adult population on HMV²⁷⁻²⁹ and 3 publications have reported the telemedicine experience in children. The use of telemedicine compared favorably to clinic visits and was acceptable to the patient and family and user-friendly,^{27,29-31} it decreased admissions, clinic visits or emergency room visits²⁷⁻³⁰ and was cost-effective.²⁸⁻²⁹

Clinical practice guidelines have suggested a global reassessment every 6 to 12 months.^{20,21} It is usually suggested that the first assessment occur in the first month and no later than 3 months after discharge home. After the first visit, the interval between assessments will depend upon the status of the child and the disease process.

Information on the optimal ways to evaluate the effectiveness of ventilation in children using HMV was available from 3 consensus statements^{21,23,24} and 2 publications have evaluated the frequency of changes in ventilator settings following recordings done in hospital or at home.^{25,26} Ideally, each home ventilator (invasive or NIV) should have downloadable memory to allow: assessments during clinic visits or at home of the effectiveness of ventilation; review of the logs of alarms, their type and frequency; and to enable reviewing of the patient's ventilator adherence. A home assessment should entail at a minimum a review of the oxygen saturations measured by pulse oximetry (SpO₂) and end-tidal or transcutaneous carbon dioxide recordings overnight. Performing a PSG for reassessment of the effectiveness of ventilation is usually recommended on a 6-month or yearly basis.²³

Recommendations for follow-up care for children using long-term mechanical ventilation at home

Given the paucity of published data and the lack of recent clinical practice guidelines, the following are consensus recommendations.

1. **The first visit and the frequency of subsequent visits should be tailored to the child and family's need. The first visit should occur within 3 months after discharge home based on patient acuity and subsequent visits should be at a minimum of every 6 months. (Consensus)**
2. **Effectiveness of ventilation should be assessed regularly. (Consensus)**
 - a. **A polysomnogram (ideal) or an assessment of oximetry and capnography, if polysomnogram is not available, should be performed yearly.**
 - b. **The home ventilator (invasive or noninvasive NIV) should be downloaded during clinic visits or at home to review the effectiveness of ventilation as well as to review the logs of alarms, their type and frequency, and to review adherence.**
3. **Quality of Life and/or other parameters of family and patient well-being should be regularly measured. (Consensus)**

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Section 5: Airway clearance in children using long-term mechanical ventilation at home

Introduction

Airway patency and adequate cough clearance are required in order to maintain adequate ventilation, but are compromised in patients with respiratory muscle weakness and those receiving invasive ventilation via tracheostomy, where adequate glottic closure is impaired. Recurrent atelectasis and pneumonia are frequent complications in these patients.^{1,2,3,4,5} Supportive airway clearance techniques⁶ assist with removal of secretions from the lungs and airways.^{3,6–9} Noninvasive airway clearance strategies (1) increase inspired lung volume to reach maximum insufflation capacity (MIC), through Lung Volume Recruitment (LVR) or “breath-stacking”, (2) increase expiratory force in the expulsive phase of cough with manually assisted coughing, and/or (3) accelerate expiratory airflow through application of positive and negative airway pressures with mechanical in-exsufflation. Physiotherapy techniques such as percussions and vibrations assist with mobilization of secretions from the distal airways. Agents that alter viscosity of secretions may aid with airway clearance. Finally, in individuals with tracheostomies, suctioning is often also applied, to clear secretions from the large airways.

Key evidence

LVR is the most commonly employed technique to assist with cough and airway clearance in individuals with respiratory muscle weakness. Insufflation may also help to maintain chest wall range of motion and lung compliance.⁷ LVR improves cough efficacy in adults and children with neuromuscular disease.^{3,10} It may be applied in individuals at risk of requiring or already receiving NIV. LVR techniques include: 1) glossopharyngeal breathing (air stacking or “frog breathing”),^{11,12} 2) insufflation with a self-inflating resuscitation bag and patient interface with a one-way valve⁶ with or without a manually assisted cough,^{7,13–15} or 3) mechanical in-exsufflation by delivering positive pressure breaths, followed by a rapid negative pressure to mimic a cough.^{1,3,16} Most studies performed to date have incorporated LVR as an integral part of an overall

approach to care, making it difficult to assess its impact alone on clinical course.¹⁷

Optimal frequency of usage of LVR and mechanical in-exsufflation is not known, although it has been shown that during the first 24–36 hours of an intercurrent illness, individuals with weak respiratory muscles experience a decline in pulmonary function¹⁸ and airway clearance techniques are therefore critical at these times to prevent morbidity and mortality. The patient populations who will benefit most from these interventions have not been clearly determined. Lung and chest wall compliance, as well as bulbar function determine the degree to which LVR can be performed.^{17,19} LVR techniques may be most applicable to patients with neuromuscular or chest wall disease but may also benefit those with obstructive airways diseases.²⁰

LVR has been recommended by Bach and Finder as the “standard of care” for neuromuscular patients.^{1,9,17,21–25} Although thresholds of FVC < 40–60% predicted,^{9,26} peak cough flow (PCF), and maximum expiratory pressure have been suggested as guidelines for the initiation of LVR, ranges provided are for adults and cannot necessarily be applied to younger children.^{9,25}

There are no comprehensive, prospective long-term studies on the use of LVR in ventilator-assisted children and youth. Most studies were performed primarily in adult patients,²⁷ although some included small numbers of children with neuromuscular disease. Of these studies, 3 looked at the effect of mechanical in-exsufflation. One case series, which studied mainly adults, demonstrated an improvement in MIC, despite a decrease in vital capacity, over 0.5–24 years of follow-up in 282 patients with neuromuscular disease.²⁸ A similar protocol using noninvasive positive pressure ventilation (NIV) and LVR has been used in a prospective cohort study to avoid intubation and death in episodes of acute respiratory failure in 79.2% of the cohort of adults with neuromuscular disease.⁵ In a largely pediatric population there has only been 1 retrospective review of long-term regular (once a day to every 4 hours) use of mechanical in-exsufflation in 62 individuals with neuromuscular disease and impaired cough (age range 3 months to 28.6 years), with a median duration of 13.4 months²⁹ 6% of participants experienced an improvement in chronic atelectasis and 8% noted a reduction in frequency of pneumonias, although the number of acute lower respiratory tract infections was too small to permit meaningful comparison with a pre-treatment period. In an analysis of a patient registry for Spinal Muscular Atrophy Type I, the use of a mechanical in-exsufflation device had a significant independent effect in reducing death.³⁰ Excellent tolerance ($\geq 90\%$) of LVR has been reported in children.^{7,29,31} Optimal positive and negative pressure settings for manual and mechanical in-exsufflation are not known and range in the literature from ± 15 –45 cm H₂O.³² The British Thoracic Society Guidelines for Neuromuscular Disease also recommend that mechanical in-exsufflation should be considered in very weak children, those with loss of bulbar function and those who cannot cooperate with manual lung volume recruitment techniques or in whom the methods are not effective.⁴⁴

The use of Chest Physiotherapy techniques, including High Frequency Chest Wall Oscillation (HFCWO) and Intra-

Pulmonary Percussive Ventilation (IPPV) are controversial and not fully established in individuals with respiratory muscle weakness.^{6,9,33–38} Similarly, agents that alter viscosity of secretions have not been well studied in children with impaired cough. On a case-by-case basis, in those with atelectasis, such treatments may be considered.⁶ Finally, management of associated comorbidities of the underlying condition that contribute to impaired airway clearance need to be addressed, including obesity, aspiration of oral secretions and/or food, as well as gastroesophageal reflux.⁶

For individuals with artificial airways,^{39,40} suctioning via catheter is a method to remove secretions from the large airways. The mechanical in-exsufflator can be used to clear secretions from the peripheral airways. Additional adjunctive treatments to enhance airway clearance in this population include increasing patient mobility and repositioning,^{39,41} and the use of heated humidity.^{39,40,42} Tracheostomized patients also require adequate fluid intake to avoid dehydration, which can result in inspissated secretions. Uncuffed tracheostomy tubes are preferred over cuffed tubes if ventilation can be provided adequately.

Conclusions

Manual and mechanical in-exsufflation for airway clearance have been shown in observational and cohort studies to have benefits in individuals with impaired cough. There is a dearth of pediatric literature in this area. However, there are no randomized controlled trials to guide clinical care. While LVR has clear benefits in acute illness, its optimal role and applications in routine care remain unknown, particularly in invasively ventilated patients. Similarly, additional airway clearance modalities, including IPPV and HFCWO are not well studied, but may be beneficial in select patients. Suctioning and mechanical in-exsufflation for some patients remain the standard of care for airway clearance in tracheostomized individuals.

Recommendations for airway clearance in children using long-term mechanical ventilation at home

Patients using NIV

1. **Airway clearance techniques should be taught to children and caregivers as a preventative strategy in those with evidence of impaired cough, especially if they have had episodes of deterioration with respiratory infections. (GRADE 1C)**
2. **In the absence of contraindications,* manual and/or mechanical lung volume recruitment techniques should be introduced for children with impaired cough (defined by clinical assessment, and/or MEP < 60 cm H₂O and/or PCF in children ≥ 12 years** < 270 L/min and/or FVC < 40% predicted. (GRADE 2C)**
3. **Mechanical lung volume recruitment techniques (ie, mechanical in-exsufflation) should be considered in very weak children, those with loss of bulbar function and those who cannot cooperate with manual lung volume recruitment techniques or in whom the methods are not effective. (GRADE 1C)**

4. **High Frequency Chest Wall Oscillation (HFCWO) and Intra-Pulmonary Percussive Ventilation (IPPV) could be considered for patients with impaired cough with atelectasis/consolidation, despite use of other airway clearance techniques. (Consensus)**

Patients ventilated via tracheostomy

1. **Minimally invasive rather than deep suctioning is recommended when possible. (GRADE 2B)**
2. **Heated humidity is recommended over heat-humidity exchangers. (GRADE 1A)**
3. **Clean, as opposed to sterile, conditions are adequate for home secretion clearance and suctioning. (GRADE 2C)**
4. **Mechanical in-exsufflation and manual LVR for tracheostomy airway clearance should be considered through the tracheostomy to complement deep suctioning. (GRADE 2C)**
5. **Adequate hydration (optimized fluid intake) is essential to maintain thin, easily cleared secretions, especially in infants. (GRADE 2C)**
6. **High Frequency Chest Wall Oscillation (HFCWO) and Intra-Pulmonary Percussive Ventilation (IPPV) could be considered for patients with impaired cough with atelectasis/consolidation, despite use of other airway clearance techniques. (Consensus)**
7. **Mechanical lung volume recruitment (ie, Mechanical in-exsufflation) should be available in the acute setting in all hospitals that treat children using HMV with the purpose of preventing deterioration. (Grade 2C).**

*Contraindications for manual/mechanical lung volume recruitment techniques see <http://www.irrd.ca/education.43>

**Peak cough flow in healthy children < 12 years of age may be < 270L/min.²⁶

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Section 6: Long-term mechanical ventilation at home in chronic lung disease

Introduction

HMV has been used with increasing frequency over the last 25 years among children with chronic lung diseases such as bronchopulmonary dysplasia (BPD) and cystic fibrosis (CF). There are no randomized controlled trials of HMV in either population. The following summary is based primarily on retrospective, observational studies and expert consensus.

Key evidence

Bronchopulmonary dysplasia

In Canada there is growing recognition that HMV through a tracheostomy is feasible and can be achieved safely in children with BPD. Studies in the US and Europe have observed survival of 81%–95%^{1–3} among these children, with 67%–79% being successfully weaned from ventilation before age 5 years.^{1,3} Outcomes are better among children without concurrent additional congenital heart disease or central nervous system insults.^{2,4}

The perceived benefits of HMV for children with BPD, include an earlier discharge from hospital, and possible improvement in social and neurodevelopment for the child as well as overall quality of life (QoL),⁵ but there is little evidence to support these notions. Furthermore, there is a paucity of studies to determine the optimal timing for consideration of tracheostomy in these children. HMV has been shown to reduce health care costs^{6,7} for children with BPD in other countries, but this has not been studied in Canada.

Though there is no consensus on the best type of home ventilator to use in infants with BPD, it is recommended that patient-specific adjustments to ventilator parameters should be made, based on work of breathing and gas-exchange, as measured through capillary blood gases, pulse oximetry, and end-tidal carbon dioxide (CO₂) monitoring.⁸ There is little published on specific techniques for weaning of HMV but consensus recommendations include regular monitoring of oxygen saturation, end-tidal CO₂, and capillary blood gases, maintenance of growth and activity, as well as routine screening for pulmonary hypertension during weaning.^{7,8}

Cystic fibrosis

The efficacy of NIV in CF patients remains controversial as the evidence remains mixed. Most studies of NIV outcomes in CF involve samples including both adult and pediatric patients. Such studies have demonstrated that nocturnal nasal bilevel intermittent positive pressure ventilation is well tolerated, unloads respiratory muscles,⁹ reduces respiratory rate, improves quality of sleep and increases capacity to perform activities of daily living.¹⁰ In addition to symptom management, NIV may also stabilize lung function decline.^{11,12}

There is evidence that NIV improves respiratory acidosis and dyspnea,^{13–15} and a recent Cochrane review concluded that in CF patients with moderate to severe disease, NIV, in addition to oxygen, may improve gas exchange more than oxygen alone.¹⁶

There is no consensus on when to initiate NIV in CF patients, though pulmonary exacerbation is the most common indication, followed by stable, persistent diurnal hypercapnia. NIV has been used in adult CF patients in chronic respiratory failure as a bridge to transplant, thereby improving survival once lung transplantation occurs, and one pediatric retrospective cohort study reported on 6 children who were using NIV just prior to successful lung transplantation.¹²

Conclusion

There is evidence to suggest that HMV for children with chronic lung diseases can be used safely and is well tolerated. While delivery of ventilation through a tracheostomy is recommended for infants with BPD, nocturnal NIV is the preferred mode for children with CF. More pediatric-specific studies are needed to identify the best timing for initiation of HMV for children with chronic lung disease, how best to titrate or wean support, as well as the long-term health benefits of both NIV and invasive ventilation at home in children.

Recommendations for long-term mechanical ventilation at home in chronic lung disease

1. Consider tracheostomy and HMV in children with bronchopulmonary dysplasia with stable pressure settings that are achievable with a home ventilator, with an FiO₂ < 0.4 and children who are otherwise medically stable, demonstrating stable growth, and can be safely transported. (Grade 1C)
2. Children with bronchopulmonary dysplasia who are being considered for home ventilation should be discharged with a tracheostomy + ventilator (rather than NIV). Adequacy of ventilation should be determined before discharge based on work of breathing and optimal gas exchange as measured through capillary blood gases, pulse oximetry, and end-tidal CO₂ monitoring. (Grade 1C)
3. Weaning of home ventilation should be considered once oxygen saturations are consistently > 95% and the child is demonstrating growth and health stability. (Grade 1C)
4. Home NIV should be considered in Cystic Fibrosis patients who have evidence of any of the following: sleep-disordered breathing (SDB), hypercapnia, nocturnal desaturations, increased work of breathing, poor exercise tolerance, and a decline in FEV₁ to below < 30% predicted. (Grade 1C)
5. Efficacy of NIV in a Cystic Fibrosis patient can be evaluated by blood gases, nocturnal oximetry, PSG, exercise tolerance, patient comfort, and quality of life. (Grade 1C)

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Section 7: Long-term mechanical ventilation at home in spinal muscular atrophy

Introduction

Spinal Muscular Atrophy (SMA) is a recessively inherited neurodegenerative disorder of the anterior horn cells of the spine.¹ The International Spinal Muscular Atrophy Consortium (ISMAC) in 1992 established the presence of 3 types of SMA characterized by varying clinical severity²: Type I (also known as Werdnig-Hoffman disease), is diagnosed between birth and 6 months of age and defined by the inability to sit independently. The majority of such patients have a life expectancy < 2 years.³ The onset of SMA Type 2 is before 18 months. Patients are able to sit independently but will never stand; death generally occurs after the age of 2.² Type 3, is the mildest form of the disease with onset after 18 months of age with affected children being able to stand and walk independently; life expectancy is normal.⁶

Key evidence

There are no randomized controlled trials of ventilatory management for children with SMA in the pediatric literature. The following is based on prospective or retrospective case control and cohort studies with the majority of the evidence focusing on various aspects of NIV.

NIV

The goals of NIV include providing improved quality of life (QoL) for patients and families, adequate inspiratory chest wall expansion, normalization of nocturnal and diurnal gas exchange, prevention of respiratory infection, hospitalization, and improvement of overall survival.^{4–15} There have been 5 studies assessing the efficacy of NIV for the treatment of SDB in children with SMA.^{5–9} Improvements in the respiratory disturbance index and sleep architecture have been reported in patients. Symptomatic benefits in sleep quality, headaches, daytime sleepiness, appetite, concentration, and sweating have also been noted.⁵ NIV has been shown to reduce hospital as well as intensive care unit stays.⁷ There were 85% fewer days in hospital and 68% less ICU days in the year following NIV initiation as compared to the preceding year in a mixed population of 15 children with neuromuscular conditions, some of whom had SMA. Although children with SMA Type 1 on NIV required

multiple admissions during infancy, they had fewer admissions with increasing age.^{8,10–11} Registry data indicate that ventilation (invasive or noninvasive) for greater than 16 hours per day in addition to use of the mechanical in-exsufflation (MI-E) device and gastrostomy tube feeds are the 3 factors that significantly improve survival after controlling for demographic and clinical care variables.³ In the group of patients with later presentation, improved survival on NIV is seen; in some cases, NIV can ameliorate symptoms as well as extend life.^{3,8,10–11} The use of NIV in SMA is generally considered safe, when used with adequate pressures and correct mask placement, and has been shown to have no deleterious effects on patient hemodynamics.¹³ However, potential limitations to NIV include difficulty identifying a well-fitting interface, particularly for infants, as well as skin irritation and/or breakdown, and midface hypoplasia. In addition, gastric distension and emesis are recognized risks, which can subsequently lead to aspiration pneumonia and even death.¹⁶

During acute respiratory illness, patients experience an increased ventilatory load, increased muscle weakness and ineffective secretion clearance leading to ventilatory decompensation¹ as well as difficulties in oxygenation secondary to secretions and mucus plugging. Therefore, the use of NIV plus supplemental oxygen to bridge periods of respiratory illness may be helpful. If nocturnal NIV is already in place then a transition to continuous use may be needed; short-term daytime NIV may further help augment the effectiveness of chest physiotherapy and airway clearance. Given the progressive nature of the disease, it is not uncommon for acute illness to lead to intubation, tracheostomy or death, especially for children with SMA Type 1. However, in a retrospective study, Bach et al reported a noninvasive respiratory management approach for respiratory failure in children with SMA Type 1.¹¹ Using a protocol that entails targeting room air oxygen saturations greater than 94% prior to extubation attempts, using the MI-E device during and post extubation, and using NIV and airway clearance rather than supplemental oxygen alone when oxygen saturations dropped to less than 94% post extubation, only 1 of 11 patients required a tracheostomy following unsuccessful extubation attempts.¹¹

NIV can also be used as a palliative care adjunct to facilitate discharge home from hospital or to reduce the work of breathing. Birnkrant et al. published the first case series of 4 children with SMA Type 1 for whom NIV was initiated with a palliative indication after the development of aspiration pneumonia.¹⁷ Although duration of survival was only 1–3 months after the initiation of NIV, it facilitated the ability to care for these children at home.¹⁷ Two cohort studies, one prospective and one retrospective, have since reported on the palliative application of NIV.^{13,18} In these studies it is suggested that the use of NIV in the subset of children with SMA, severe neuromuscular weakness, and evidence of bulbar dysfunction, appeared to transiently improve QoL for patients and their families, and facilitate transfer home.

Although NIV is not routinely used as a prophylactic measure in other neuromuscular diseases, there is some interest in using NIV this way in infants with SMA Type 1. It has been suggested based on case reports of infants with SMA Type 1 who demonstrate paradoxical breathing that “high span” NIV

(inspiratory pressure ≥ 10 cm H₂O above expiratory pressure) has the potential to reduce the pectus excavatum deformity, improve chest wall development¹⁹ and normalize inspiratory muscle synchrony.⁹

Tracheostomy and invasive ventilation

There are no randomized controlled trials evaluating the outcomes of children with SMA managed with invasive ventilation, but cohort studies demonstrate that long-term survival is possible for children with SMA who are invasively ventilated.^{1,4} Invasive ventilation offers the advantage of providing a secure airway, access for pulmonary toileting through a tracheostomy, as well as support of ventilation, particularly if high ventilator settings are required. However, the burden of care for families is tremendous as the majority of patients are discharged to the home environment. One potential complication of a tracheostomy for SMA patients is the loss of speech. It has been described that the tracheostomy process may lead to weakness of the bulbar muscles resulting in an inability to phonate or make sounds, and therefore an inability to verbally communicate despite average or above average intelligence.⁸ Bach et al. reported only 1 of 16 patients retained comprehensible speech after tracheostomy insertion and similarly, only 1 maintained the ability to spontaneously breathe.¹¹ However, more recent reviews suggest that speech can be retained in some children after tracheostomy insertion.^{20,21} Invasive ventilation via tracheostomy may limit socialization and the attendance of school without the appropriate resources.

Conclusion

There has been a significant improvement in survival in recent years for children with SMA due to the introduction of ventilatory support, airway clearance with the MI-E device, and aggressive nutritional management. In particular, there has been an increase in the number of patients treated with NIV. However, despite improved survival, an appreciation for QoL must always be tempered with the ability to prolong life, and prognosis, based on SMA type. Special considerations should be given when counseling families about ventilation for their children with SMA to the type of SMA (1, 2 or 3) as well as the age of diagnosis as these 2 factors have been shown to affect outcomes. The advantages and disadvantages of the different ventilation modalities must also be presented. Ultimately, the role of the treating physician is to present all of the therapeutic options, offer recommendations, and support the family through the decision-making process. In clinical practice, the parent's authority is recognized when there is more than one option for treatment.

Recommendations for long-term mechanical ventilation at home in spinal muscular atrophy

1. **Children with SMA whose parents would like a respiratory opinion should be referred to an experienced center to discuss the treatment options. (Grade 1C)**
2. **The option of invasive and noninvasive HMV support as well as the differences between therapeutic and**

palliative NIV should be presented to all parents of children with SMA. (Grade 1C)

3. **Assessment for sleep disordered breathing (ideally with a polysomnogram conducted as per the American Academy of Sleep Medicine standards) should be considered for children with SMA if this is in accordance with the child and family's goals of care. (Grade 1C)**
4. **The decision to recommend or not recommend ventilatory support to children with Type 1 and Type 2 SMA by the treating physician should be made on a case-by-case basis after discussion with family and other caregivers, and a careful assessment of medical benefit as well as the effect on quality of life. (Grade 1C)**
5. **Airway clearance is an important adjunct to ventilator management in children with SMA. See Section 5 recommendations. (Grade 1C)**
6. **If children are intubated, a protocol-led extubation, including the use of a mechanical in-exsufflation device and NIV, is recommended. (Grade 1C)**
7. **When and how to augment ventilatory support during acute illnesses should be made on a case-by-case basis for each patient by the treating physician, as there is no validated protocol at present. (Grade 1C)**

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Section 8: Long-term mechanical ventilation at home in muscular dystrophies

Introduction

Duchenne Muscular Dystrophy

Duchenne Muscular Dystrophy (DMD) is the most common muscular dystrophy and is present in about 1 in 3,600 to 6,000 newborn males.¹ The natural history of the disease is for lung function to peak during the pre-adolescent period² and then decline due to muscle weakness by approximately 5–8% each year in the adolescence^{3–8} with eventual diurnal hypercapnia by late adolescence and death typically from respiratory failure and death by age 20.⁹ However, the progression of DMD has been favorably modified by medical treatments including systemic corticosteroids and cardiac disease specific medications.¹

Congenital Muscular Dystrophies

Congenital Muscular Dystrophies (CMD) are a heterogeneous group of diseases that present with muscle weakness and/or hypotonia within the first few months of life, but have variable courses of disease progression. From a respiratory point of view, patients may vary from needing ventilation in the neonatal period such as with A-Dystroglycan deficiency CMD¹⁰ to rarely requiring any supports before the fifth decade of life such as with mild forms of Congenital Muscular Dystrophy Type 1A.¹¹ In certain CMD such as Rigid Spine CMD, the need for nocturnal ventilation may predate the loss of ambulation.¹²

Key evidence

Benefit of long-term home ventilation

There is a lack of well-designed randomized control studies evaluating the effect of long-term ventilation on survival in children with DMD or other neuromuscular diseases. In one study, “pre-emptive” initiation of NIV in DMD was reported to lead to an increased mortality, however this study has been criticized for a number of methodological flaws.^{13–15} A second small RCT with a heterogeneous neuromuscular population showed no difference in survival. However 9 out of 10 individuals in the control group developed daytime hypercapnia during the 24-month follow-up period and were started on NIV.¹⁶

Other studies have shown that the use of NIV improves gas exchange abnormalities and normalizes blood carbon dioxide (CO₂) levels.^{17–19}

Most evidence for the benefit of nocturnal ventilation comes from historical cohort comparative studies, from multiple centers around the world predominately with NIV. These demonstrated survival into the third to fifth decades of life with long-term ventilation.^{9,20–24} This has been maintained while maintaining QoL.^{19,25–27} At present, given the large increase in survival attributable to NIV, it would not be ethical to further evaluate NIV with new randomized control trials. There is also emerging evidence from multiple centers that daytime hypercapnia can be managed effectively with nocturnal NIV in addition to daytime mouthpiece ventilation.^{9,28–31} However a subset of patients may still prefer or require tracheostomies, particularly if they lack adequate neck and/or oral control.³²

Recognition of nocturnal hypoventilation

Nocturnal hypoventilation predates daytime gas exchange abnormalities and individuals are usually asymptomatic. As such symptom questionnaires and daytime blood gases are not sensitive screening tests for SDB, which includes obstructive sleep apnea, central sleep apnea, and/or nocturnal hypoventilation.^{33–36} Nevertheless when blood gas abnormalities are present, they can be quite specific and indicate the need for further evaluation. In one study, the presence of elevated base excess > 4 was 100% specific for nocturnal hypoventilation, however in this study it only had a sensitivity of 55%.³⁵ An abnormal daytime pCO₂ predicts nocturnal hypoventilation, however this is a late sign that may be associated with increased mortality.^{36,37}

Vital Capacity remains the best-correlated test with nocturnal hypoventilation. A decrease in forced vital capacity (FVC) correlates with an increased risk of hypoventilation and need for ventilatory support. Several studies of adolescents and young adults with DMD and other Neuromuscular Disorders have demonstrated that low vital capacities of < 35–40% are very sensitive for continuous nocturnal hypoventilation and the need of nocturnal ventilation, however patients may be quite symptomatic and may have daytime hypercapnia.^{38–40} However in at least one other study, patients with DMD already had daytime hypercapnia with an FVC in the 40–50% predicted range.⁴¹ In more recent studies in mixed groups of adults with neuromuscular myopathies, an FVC < 60% was approximately 90% sensitive and specific for the onset of sleep disordered hypopneas (REM related hypopneas) whereas an FVC < 40% was 94% sensitive (79% specific) for continuous nocturnal hypoventilation.^{35,42} In a Canadian study in children with a heterogeneous group of children with neuromuscular disease, Katz et al. found that FVC of less than 70% predicted had a sensitivity of 71.4% for nocturnal hypoventilation.³⁴ Given that dystrophinopathies are associated with cardiomyopathies and cardiac death, earlier recognition of SDB would be important. Maximal respiratory muscle testing including Maximal Inspiratory Pressures (MIP), Maximal Expiratory Pressures (MEP), and Sniff Nasal Inspiratory have a weaker correlation than Vital Capacity for nocturnal hypoventilation.^{34–36,40,42,43}

At present, there is no international consensus as to when to screen for SDB in children with neuromuscular disease. The published recommendations in the field include the following. The 2004 American Thoracic Society consensus statement recommends that, where available, annual PSG testing would be ideal for surveillance. Alternatively overnight oximetry and capnography can be performed.⁴⁴ The 2010 DMD Care Considerations working group recommends nocturnal evaluation of gas exchange when there are signs and/or symptoms of hypoventilation, a baseline FVC < 40% predicted, and/or daytime hypercapnia with $p\text{CO}_2 > 45$ mmHg or with a room air oxygen saturation < 95%.⁴⁵ The 2011 Canadian Thoracic Society *Adult Home Ventilation Guidelines* for the same population suggest performing an evaluation for nocturnal ventilation if there are clinical signs of hypoventilation and/or if the FEV₁ or FVC is < 40% predicted.¹⁵ The 2012 British Thoracic Society *Neuromuscular Guidelines* recommend annual nocturnal assessment with PSG or oximetry when there is a vital capacity < 60% predicted or a loss of ambulation. It also suggests considering annual testing in infants with neuromuscular weakness, children with diaphragmatic dysfunction, Rigid Spine Syndrome, and/or when there are symptoms of hypoventilation.⁴⁶ Polysomnograms should be conducted in accordance with the American Academy of Sleep Medicine recommendations.⁴⁷ If a polysomnogram is not available for annual surveillance, oximetry with capnography (etCO₂ and/or tcCO₂) is recommended.

Conclusions

The initiation of long-term home ventilation can lead to prolongation of life in DMD, while maintaining QoL. The evidence for CMD is less robust, but can be extrapolated from the DMD literature. The primary challenge in the pediatric age group range is knowing when and how to recognize SDB. Decreasing vital capacity is associated with an increased risk of nocturnal hypoventilation and the need for ventilatory support. The evidence supports that those with a FVC (or FEV₁) < 40% predicted and/or those who have daytime symptoms of hypoventilation should be screened for signs of SDB, ideally with PSG, as they are at the highest risk of hypoventilation and hypercapnia. This approach will likely miss individuals with early and milder SDB. An FVC < 60% predicted cutoff is likely to be a better threshold for the initiation of screening for nocturnal hypoventilation. Regardless of the vital capacity, if the patient is symptomatic or has an abnormal blood gas they should also undergo evaluation. As the disease progresses and daytime hypoventilation develops, ventilatory support up to 24 hours a day can be provided by both invasive and noninvasive methods. Noninvasive methods may provide the advantage of decreased secretions and hospitalizations. Especially in the DMD population, candidacy for NIV plus mouthpiece ventilation to manage diurnal hypercapnia should be evaluated prior to tracheostomy insertion.

Recommendations for long-term mechanical ventilation at home in muscular dystrophies

1. *Assessment for sleep disordered breathing, ideally with a polysomnogram, should be carried out not less than annually for children with a FVC < 60% predicted and/or who have symptoms of sleep disordered breathing. (Grade 1C)*
2. *Assessments for sleep disordered breathing, ideally with a polysomnogram, should be considered annually in:*
 - a. *Infants with congenital muscular dystrophies demonstrating weakness.*
 - b. *Infants with congenital muscular dystrophies that may develop hypoventilation < 5 years of age (including severe forms of MDC1A and A-Dystroglycan deficiency congenital muscular dystrophy CMD).*
 - c. *Children with congenital muscular dystrophies who never attain the ability to walk or become nonambulant. (Consensus)*
3. *If a polysomnogram is not available for annual surveillance, oximetry and capnography (etCO₂ and/or tcCO₂) are recommended. (Grade 1C)*
4. *Children who have shown a clinical deterioration, who are having recurrent pulmonary exacerbations, and/or who develop symptoms of sleep disordered breathing may require sleep assessment more often than annually. (Consensus)*
5. *Initiation of NIV should be considered when there is evidence of diurnal hypoventilation (daytime $p\text{CO}_2 \geq 45\text{mmHg}$) and/or nocturnal hypoventilation (tcCO₂/etCO₂ > 50mmHg for > 25% of the total sleep time) with symptoms. (Grade 1B)*
6. *Initiation of NIV should be considered in patients with evidence of SDB (hypoventilation, obstructive sleep apnea, and/or central sleep apnea), even if asymptomatic. (Grade 1C)*
7. *For patients who require > 12 hours of ventilation a day, noninvasive supports with mouth piece ventilation should be considered as a possible alternative to tracheostomy and continuous mask NIV, factoring in individual circumstances and preferences. (Grade 1C).*

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Section 9: Long-term mechanical ventilation at home in children with kyphoscoliosis

Introduction

Kyphoscoliosis is a well-recognized cause of respiratory failure in adults.¹ In pediatrics, there is a growing body of literature on respiratory failure secondary to early onset scoliosis as well as scoliosis associated with other bony abnormalities of the thorax. Surgical spinal stabilization is an integral component of the treatment of scoliosis, and is performed to preserve respiratory function, to prevent the future development of pulmonary hypertension, to improve quality of life by facilitating seating, and for cosmetic reasons (to improve symmetry on standing).^{2,3}

Key evidence

Provision of HMV to patients with respiratory failure and scoliosis is associated with long-term benefits

There were no studies specific to pediatric patients either receiving HMV as a consequence of isolated scoliosis, or in association with neuromuscular diseases.

Predictors of prolonged mechanical ventilation post-surgical scoliosis repair

We found 11 retrospective studies reporting on 592 pediatric patients undergoing spinal fusion surgery of which 134/558 (24%) required prolonged mechanical ventilation, defined as ≥ 36 hours.^{4–14} All of the studies included children with preoperative Forced Vital Capacity (FVC) percent predicted less than 30–40%. Three studies found that lower preoperative FVC % predicted significantly predicted the need for prolonged mechanical ventilation.⁵ However, the precise values of FVC that predict the need for postoperative ventilation varied considerably between studies, and ranged from 34 to 60; all 3 of these studies incorporated different FVC cutoffs. Jenkins et al. found that prolonged mechanical ventilation only occurred in patients with FVC less than 40% predicted.^{21,6–7} Udink et al. reported the mean (range) FVC % predicted in those with prolonged MV was 34% (21–25%) as compared to 62% (30–114)% in those not needing prolonged MV.⁶ Finally, Yuan et al. found that FVC < 60% predicted was the most significant predictor of prolonged mechanical ventilation.⁷ Two studies did not find that FVC was a significant predictor of prolonged MV; and similarly 2 studies found that a lower Forced Expiratory Volume in one second (FEV₁) significantly predicted the need for prolonged mechanical ventilation.^{6,7,12} In those patients unable to perform pulmonary function tests, one retrospective study reported on the use of infant pulmonary function testing and PSG to predict the need for mechanical ventilation post spinal fusion, but found no significant relationship.¹³ Other predictors of prolonged mechanical ventilation reported to be significant by Yuan et al. included older age, male gender, and neuromuscular scoliosis etiology.⁷ These findings were not reproduced by other studies. Zhang et al. found surgical approach to be the only predictor of prolonged mechanical ventilation in their study, while Gurajala et al. found longer vertebral fusions and hypothermia to be associated with prolonged mechanical ventilation.^{12,14}

Pulmonary function post-surgical scoliosis repair

Pulmonary function following scoliosis surgery has been studied comprehensively in patients with idiopathic scoliosis but not in patients with neuromuscular scoliosis.^{15–27} Following scoliosis surgery, pulmonary function decreases dramatically in the immediate postoperative period, although it improves during the weeks following surgery, with resolution of postoperative secretion retention and atelectasis.¹⁹ At 3 months following surgery, many patients will have returned to baseline values, and most will reach their preoperative baseline by 2 years postoperatively.^{15,17} However, many patients, particularly those whose surgery included thoracotomy and those patients with more advanced restrictive lung disease, may have long-term reductions in pulmonary function.²²

New surgical techniques incorporating thoracoscopic anterior approaches, may reduce morbidity and have less detrimental effects on pulmonary function.^{20–22} More recent studies have demonstrated possible improvement in lung function at 2- and 3-year follow-up.^{26,27} Huitema et al. demonstrated a significant improvement in FEV₁ in 20 adolescents with scoliosis at 2-year follow-up, as compared to baseline.²⁶ Demura et al. demonstrated similar results in a prospective cohort of 154 adolescents.²⁷ However, the increases in absolute lung function values that are reported are likely due to growth rather than true improvement. There was no change in FEV₁% predicted.

Reduction of morbidity post-surgical scoliosis repair

The most consistent predictor was FVC % predicted. In general, the severity of the restrictive lung disease as measured by preoperative pulmonary function tests (PFTs) appeared to correlate with the need for prolonged mechanical ventilation in the postoperative period.

Scoliosis patients who have not previously been on assisted ventilation may benefit from NIV following extubation.^{15,19} Since the introduction of postoperative NIV, failure to wean from invasive ventilation is rare. Most patients not receiving assisted ventilation prior to surgical correction will be able to discontinue NIV prior to hospital discharge, although a small proportion may need to continue on assisted ventilation at home. Preoperative NIV and assisted cough techniques, in addition to routine extubation to NIV post procedure will result in a reduced incidence of failure to wean from invasive ventilation, and may shorten hospital stay. See Section 5 on Airway Clearance Techniques for further details.

Conclusions

In summary, the provision of HMV to patients with respiratory failure and scoliosis is associated with long-term benefits. From the available literature, a meaningful estimate of the percentage of patients requiring mechanical ventilation postoperatively following scoliosis repair based on FVC % predicted cannot be determined. However, what does seem to emerge clearly from the literature is that a mandatory preoperative tracheostomy is no longer essential, given the improved preoperative care, surgical techniques, and outcomes. This does not preclude a frank discussion with the patient and family regarding the risks of spinal surgery including the possibility of prolonged ventilation using NIV or tracheostomy in at-risk patients. It is also apparent that despite the effects of stabilization on the spine as a result of spinal fusions, some patients may need to be maintained on assisted ventilation at home.

Recommendations for long-term mechanical ventilation at home in children with kyphoscoliosis

1. **Long-term mechanical ventilation is recommended for all patients with kyphoscoliosis who develop chronic respiratory failure. (GRADE 1C)**
2. **Preoperative pulmonary function inversely correlates with the need for prolonged mechanical ventilation post spinal fusion therapy. However, there is no consensus on a definitive FVC % predicted or FEV₁% predicted**

which predicts the need for prolonged postoperative mechanical ventilation. (GRADE 2C)

3. *Children should not be refused corrective scoliosis surgery on the basis of pulmonary function testing alone. (GRADE 2C)*
4. *A tracheostomy is not mandatory for spinal fusion surgery. (GRADE 2C)*
5. *The initiation of NIV should be considered in the preoperative period. (GRADE 2C)*
6. *There should be a low threshold for the initiation of NIV after extubation following spinal corrective surgery. (GRADE 1C)*
7. *Airway clearance techniques (breathstacking with and without assisted cough or mechanical in-exsufflation) should be provided in the postoperative period following complex spinal surgery in patients with neuromuscular scoliosis. (GRADE 1C).*

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Section 10: Obesity hypoventilation in children

Introduction

Central hypoventilation is addressed in Section 11. This section will focus specifically on hypoventilation associated with obesity, also known as obesity hypoventilation syndrome (OHS). OHS may develop either as an isolated disorder, or as part of a constellation of genetically related and inherited dysregulation syndromes.

The definition of OHS in adults has been described.¹ There is no evidence to support any specific diagnostic criteria in children, other than that used in adults:

1. Obesity (BMI > 30 kg/m²) or BMI > 95 percentile for age and gender or weight > 95 percentile for age.
2. Daytime hypercapnia (PaCO₂ > 45 mmHg).
3. Absence of known neurological, cardiac or pulmonary causes of hypoventilation.

The prevalence of OHS in pediatric patients, and the number receiving HMV, is unknown. Approximately 10–20% of

adults with obstructive sleep apnea (OSA) are estimated to have OHS.^{2,3} Around 1–4% of children are estimated to have OSA,⁴ with higher rates in children with obesity. If 1% of that group has OHS, there are still substantial numbers of children with undiagnosed OHS. Pediatric long-term home mechanical ventilation has increased substantially, but how many are treated for OHS is unclear.⁵

One study attempted to evaluate OHS in otherwise healthy children.⁶ Of 326 children, 28% were obese, but there was overlap with sleep disordered breathing (SDB). The inability to readily identify those affected with OHS is at least partially responsible for the absence of good epidemiological data on the prevalence of OHS among children.

Key evidence

A systematic search of the literature failed to find any studies that reported specifically on OHS in children.

Conclusion

The phenotype of OHS in children has not been well characterized. Clinical presentation of OHS can range from asymptomatic to presenting with acute respiratory failure. Other children may present with indolent symptoms (snoring, apneas, headaches, etc.), daytime sleepiness or hypersomnolence, altered cognitive function, and associated left or right heart failure.⁷ The diagnosis of OHS should be considered in any morbidly obese child with symptoms of SDB, somnolence, or exercise limitation, especially if daytime hypoxemia and hypercarbia are present, other causes of hypoventilation being ruled out.

OHS may involve any combination of the following: morbid obesity, altered ventilatory response, alteration of chest wall mechanics resulting from obesity, upper airway dysfunction, altered neuromuscular tone, with contributions from neurohormonal influences of leptin.^{8,9} It is unclear whether this represents a primary defect in the neurological control of breathing or is a result of habituation of the brainstem centers to hypoxemia and hypercarbia.^{8,9}

In adults, OHS subjects have an increased mortality, increased hospitalization, poorer health-related QoL, decreased vigilance, and an increased incidence of heart failure, angina, and pulmonary hypertension.^{1,10} Comparable pediatric data is not available. Whether this is also true in children has not been confirmed. QoL has been reported as improved by both caregiver and child in obese children treated with positive airway pressure therapy, but OHS was not specifically evaluated.¹¹ However, this study was not designed to specifically evaluate children with OHS.

In the adult literature there is evidence to suggest that treatment for OHS improves outcomes⁹ though only a small portion obtains treatment.¹² No pediatric data are available in studies that have looked at OHS specifically for treatment or to evaluate outcomes. When OHS has been evaluated, it is often within a heterogeneous group of individuals. The health care costs and benefits of NIV in children with OHS are not known.

OHS is likely an underrecognized cause of morbidity in obese children. Evidence extrapolated from adults suggests that

positive airway pressure therapy may be effective for children with OHS. There is uncertainty about the timing of initiation of ventilatory support and the outcomes related to the treatment. In general, NIV can be initiated in a PSG laboratory or in hospital. Many questions remain unanswered, both clinically and from a research perspective, making the care for these children challenging.

Recommendations for obesity hypoventilation in children

1. *All children with obesity should receive counseling regarding nutrition, the importance of exercise, the health care risks associated with obesity as well as the importance of a healthy lifestyle and body weight. (Consensus)*
2. *In obese children (as defined above), especially those who are symptomatic with daytime somnolence, evaluation for SDB should be undertaken with a PSG. (Grade 1C)*
3. *Obesity Hypoventilation Syndrome is a diagnosis of exclusion and other causes of central hypoventilation need to be excluded. (Grade 1C)*
4. *In obese children with OHS, NIV should be initiated. (Grade 2C)*
5. *NIV therapy can be initiated in an outpatient sleep laboratory or on a hospital ward as an inpatient. (Consensus)*
6. *Annual NIV titrations in a sleep laboratory are recommended. (Consensus)*

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Section 11: Long-term mechanical ventilation at home in children with central hypoventilation, congenital and acquired

Introduction

Congenital Central Hypoventilation Syndrome (CCHS) is a rare autosomal dominant disorder characterized by ventilatory insensitivity to hypercapnia and hypoxemia during sleep and/or wakefulness related to a mutation in PHOX2B (paired-like homeobox 2b), a transcription factor expressed in neurons.¹ CCHS may be associated with neural crest tumours, Hirschsprung's disease,^{2,3} autonomic nervous system (ANS) dysfunction, cardiac arrhythmias, esophageal dysmotility, pupil abnormalities,⁴ breath-holding spells, as well as a lack of physiologic responsiveness to exercise and environmental stressors.⁵⁻¹⁷ The primary goal of respiratory management is prevention of sleep-related respiratory failure. CCHS is a life-long condition though the degree of support required may change over time. Other causes of central hypoventilation in infants and children are usually related to a central nervous system abnormality and may be diagnosed by brain imaging.

Key evidence

There are no randomized trials examining best ventilatory practices. Most evidence comes in the form of case reports or case series. However management of CCHS has been described by experts in the field^{1,18-21} and a recent American Thoracic Society Statement on CCHS.¹

Conclusions

Chronic, lifelong ventilatory support at home is the treatment of choice for these patients because children with CCHS do not outgrow their symptoms, current pharmacologic treatments are ineffective, and oxygen therapy corrects the hypoxemia but not the ventilatory derangement.^{16,22-25} The immediate goal of ventilatory support is to achieve end-tidal CO₂ levels between 30–50 mmHg (ideally 35–40 mmHg) and oxygen saturations greater than 95%.¹⁸ The long-term goals of this management are to avoid the sequelae of recurrent hypoxemia and hypercarbia, which include adverse neurologic outcomes and pulmonary hypertension with right-sided heart failure.^{16,26,27}

Various options are available in terms of the type of support including positive pressure ventilation via tracheostomy, noninvasive positive pressure ventilation (NIV), diaphragm pacing, and negative pressure ventilation. Treatment should ideally be optimized for each individual in order to achieve normal ventilation and oxygenation as assessed by standard PSG. Anticipatory guidance and advice regarding management during intercurrent illness, avoidance of sedating medications as well as exercise-related precautions are also important considerations.

Recommendations for long-term mechanical ventilation at home in children with central hypoventilation, congenital and acquired

1. *Patients with Congenital Central Hypoventilation Syndrome should receive lifelong ventilatory support in order to avoid hypoxemia and hypercarbia. (GRADE 1C)*
2. *Ventilation should be optimized to maintain as close to normal gas exchange during wakefulness and sleep. The ideal target levels for end-tidal/transcutaneous CO₂ are 35–40 mmHg and oxygen saturations greater than 95% during sleep. (GRADE 1C)*
3. *Children with Congenital Central Hypoventilation Syndrome should undergo formal titration to optimize gas exchange (as defined in criteria in no. 2 above) at least annually by nocturnal PSG and twice a year for those under 3 years old. (GRADE 1C)*
4. *Children with other Central Hypoventilation Syndromes should undergo formal titration to optimize gas exchange (as defined in criteria in no. 2 above) at least annually by nocturnal PSG. (Grade 1C)*
5. *Children with Congenital Central Hypoventilation Syndrome should be discharged home with a portable oximeter to monitor oxygen saturations. (GRADE 2C)*
6. *The mode (positive pressure ventilation via tracheostomy, NIV, diaphragm pacing) and degree of ventilatory support should be individualized for each patient. (GRADE 1C).*

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Section 12: Transition from pediatric to adult care

Introduction

With improvements in care and technological support, increasing numbers of children on long-term ventilation are now surviving into adulthood. This has led to the appreciation of the impact of what is a major and predictable event in the child's life – transition to adult care. There is growing recognition of the requirements from both pediatric and adult health care providers to ensure all their ongoing needs (medical, emotional, and social) are met. An additional goal is the prevention of significant risks associated with a transfer: patient and family anxiety, reduction in adherence to treatment or loss to follow-up resulting deterioration in health status.

Key evidence

Although we were only able to find a few studies specifically addressing this issue for children on chronic ventilation, there is a significant literature dealing with transition of care into adulthood for many other chronic childhood illnesses.^{1–6} Position statements from the Canadian Paediatric Society and American Academy of Paediatrics, although not specifically directed toward HMV, do have sufficient commonality to inform the care of patients on HMV.^{7–9} Most of the literature in this area consists of qualitative, observational studies, and

consensus statements, with relatively little objective evidence with respect to effectiveness of differing models of transition.¹⁰

Components necessary for a successful transition

Several reviews (that did not include children on HMV) have recently reviewed existing data examining the effectiveness of various transition programs.^{1,11} They identified 10 studies that met their criteria for evaluation, 8 being for patients with diabetes mellitus, 1 for patients with cystic fibrosis, 1 for patients following transplantation and none including patients requiring ventilation. The methodology was very heterogeneous, with only 4 studies comparing a standard intervention versus a control population. Despite these limitations, there were several factors leading to a successful transition:

1. Disease specific-patient education, including self-management, preparing the patient for the transfer.
2. Creation of either specific adolescent, or joint pediatric and adult “overlap” clinics.
3. Establishment of transition coordinators with links to both programs, tasked with ensuring the transfer of the patient and necessary health care information.

Available data suggest that although all these components are required in a transition program, objective evidence would indicate that this, in fact, occurs infrequently.¹²

Issues identified that need to be considered in the transfer process include¹³:

1. Level of preparedness of the adolescent. The transition from adolescence to adulthood is a gradual process, the rate of which varies from one individual to another based on factors such as maturity and developmental age. It has been suggested that transition should not occur at a specific age, but rather anywhere between 17 and 20 years of age, depending upon the level of preparedness and maturity of the patient.⁹
2. Level of preparedness of the system. There are significant differences between pediatric versus adult-oriented health care systems, particularly in terms of the type and level of psychosocial supports, decision-making and consent processes, and family involvement.¹⁴ Furthermore as children on home ventilation frequently suffer from diverse, often previously fatal disorders, adult health care providers may have limited experience and knowledge for certain diseases.^{15,16} Ideally both the pediatric and adult clinics should be prepared to discuss issues such as sexuality and fertility, and career and employment plans.
3. Level of preparedness of the family. Parents of children on home ventilation are commonly a major participant in their child's care. Parents may therefore find difficulty in reducing their involvement to allow the adolescent the degree of independent decision making expected by most adult centers.¹⁴ This is further compounded if the child has any degree of intellectual or motor limitations impeding their ability to assume this independence.¹⁷
4. Plan of care. Explicit discussions should be undertaken to confirm the substitute decision-maker when an adolescent is unable to provide informed consent and to outline clearly for the receiving caregivers what the goals of

care are: full treatment under all circumstances or modified goals or symptom management only.

Conclusions

Transition needs to be regarded as a gradual family-centered process. Identification of providers, education, and coordination of teams is essential for successful transitioning, with no adverse sequelae on the child's treatment or health care outcomes. Several relevant guidelines are available, including the American Academy of Pediatrics, the Society for Adolescent Medicine, and the Good to Go Transition program.¹¹

Recommendations for transition from pediatric to adult care

1. **Transition planning should start early in childhood and is an ongoing process. (Consensus)**
 - a. *The patient and their current (future) caregivers should have a full understanding of their medical condition and what is required for transition into the adult health care system.*
 - b. *If feasible based on the developmental level of the child, the patient should be involved in planning discussions in a way that is meaningful to them.*
 - c. *The adolescent needs to be involved in, and assume the management of their condition prior to transition, to the extent that they are able.*
 - d. *Providers should encourage adolescents to complete developmental milestones to promote attainment of self-esteem and self-confidence, to allow successful transition, and assumption of as much independence as their condition allows.*
2. **A formalized approach to transition of care is needed. (Consensus)**
 - a. *A formal transition plan should be developed in collaboration with patient and family, by the time the patient is 14.*
 - b. *A family physician should be identified prior to transition. The patient and family are encouraged to have met with the family physician before the age of 18.*
 - c. *The formal transition program should be agreed upon and coordinated by both the pediatric and adult health care teams.*
 - d. *Joint transition clinics at either the pediatric or adult health care facility are recommended.*
3. **Plan of care. Explicit discussions should be undertaken to confirm the substitute decision-maker when an adolescent is unable to provide informed consent. Goals of care conversations should be had before transition and the results of these conversations should be clearly communicated to the adult healthcare providers. (Consensus)**
4. **The adolescent and their caregivers need to be made aware of how homecare supports will change as the adolescent transitions from the pediatric to adult health care system and how this will affect, if at all, the location of care for the adolescent. (Consensus)**

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Section 13: The published experience and outcomes of family caregivers when a child is on long-term mechanical ventilation at home

Introduction

Advances in technology and the widespread belief that children are best cared for at home have resulted in increasing numbers of children with stable chronic respiratory failure discharged on home mechanical ventilation (HMV).¹ In recent years, there has been a growing appreciation of the positive and negative consequences of the extraordinary caregiving provided by family members and other loved ones of children on HMV. This review was undertaken to promote understanding about the experiences and outcomes of family caregivers (FC) of children

on HMV, upon whom we as a society and more importantly their children rely to provide highly skilled and vigilant care in the home, up to 24 hours per day.

Key evidence

A review of the available literature regarding the experience and outcomes of family caregivers of children requiring HMV retrieved a total of 1098 studies with a total of 25 independent studies and 1 metasynthesis that met eligibility criteria. Given the studies varied highly in terms of their methods and measurement the review was presented as a narrative.

We found 1 metasynthesis and 25 individual studies that were informative. A metasynthesis by Lindahl and Lindblad² provided insight into the day-to-day experience of family caregivers of children on HMV. Major themes included siblings taking on exceptional roles and caregiving responsibilities in the home, living in an awareness of reality and important values of life, a demanding parenthood, being in a process of learning and meaning of space and place. Key themes described by these papers can be summarized and described to represent 2 major outcome types and their related outcomes: physical and mental health including QoL, caregiver burden, stress and anxiety, and social isolation, and family function, including normalization, caregiver competence, and parent/professional relationships.

Health and related outcomes

Physical and mental health. FCs of children on HMV were at a higher risk of depression, especially when supports such as care coordination and homecare nursing levels were lacking.^{3–6} Increased depression was associated with poorer sleep quality in FCs of children on HMV when compared to controls. Independent of depressive symptoms, family caregivers also reported heightened sleep disruption.^{1,8} When night nursing was not adequate, self-reported shorter sleep times were associated with greater daytime sleepiness and lower daytime functioning ($p < 0.05$).⁹ A notable limitation of these studies is the reliance on subjective measures of sleep.¹⁰ A resourcefulness training intervention was found to be acceptable and feasible in a pilot study with 22 mothers of children on HMV. In this study a 4-week, nurse-led cognitive behavioral education-based intervention demonstrated positive impacts on mental health.¹¹ In a study that targeted understanding of health promotion and lifestyle among FC of children on HMV, the healthiest scores were reported for substance use scores (eg, smoking) and safety (eg, drinking alcohol).⁸ Overall health promotion was reported to be correlated with improved functional status of the child.⁸

QoL. Qualitative studies reported that parents were happy about having their child at home.^{12,13} This appraisal was largely because parents focused on the positive aspects of their lives.^{13–15} When validated measures of QoL were used, caregivers reported negative aspects of emotional and physical health.¹⁶ More optimal physical and mental health were associated with higher scores on social support, self-esteem and psychological stability.¹⁷

Caregiver burden. Studies have described the high personal and financial costs incurred by parents who care for their child on HMV.^{2,5,12,15,16,18} Increased responsibilities, physical demands, and burden was also reported.^{5,13,15,19} One study reported that mothers felt there were demands and expectations of ‘extraordinariness’ placed on them.⁴ Having reliable family support and knowledgeable health care staff reduced the burden of care.¹⁴ Notably the use of novel communication strategies (telemedicine) saved time for the FCs.²⁰

Stress and anxiety. The technical and physical aspects of caring for the child were associated with heightened anxiety.^{15,21} Financial expenses were also associated with increased stress^{2,14,22} and increased stress was related to heightened sleep disruption.⁷ Blucker³ described that coping efforts were associated with less distress and more optimal health. In contrast, Mah²¹ found no difference in stress between FCs with children on HMV compared to controls.

Social isolation. Many families expressed increased feelings of social isolation, typically associated with not having enough home health care and community-based help.^{2,13,14,17} Notably, Miyasaka’s²⁰ pilot study showed that having a videophone for parents to access health care enabled the parents to have the unexpected benefit of gaining peer support.

Family functioning and related outcomes

Family function. There were mixed results reported for family functioning. Toly^{23,24} described that greater depressive symptoms correlated with poorer family functioning. Having the child at home also had positive effects on many families and siblings as it helped them to be more understanding and compassionate²¹ and HMV also enabled the children to be home with the family while receiving palliative care.²⁵

Normalization. Most studies found that with effort, families were able to achieve a sense of “normal” in their routine.^{13,15} Metasynthesis findings pointed however to the family home feeling like a public rather than private place.² Normalization was correlated negatively with the mother’s depressive symptoms and positively with the child’s functional status and family functioning ($p < 0.01$).²⁴

Caregiver competence. The majority of family caregivers felt confident and competent in caring for their child.^{12,14,15,19,26} Notably, the use of simulation-based education was described in one retrospective study as an important aspect of enabling confidence and competence among FC of children on HMV.²⁷

Parent/professional relationships. There were mixed feelings on the part of family caregivers about the relationships they had with professionals. Parents described that they reported conflict, lack of understanding, poor communication, and encountering inadequately prepared professionals.^{13,16,18,19,24} Parents expressed feeling comfortable and happy with the skills of home caregivers^{7,16} while adolescents on HMV in one study rated their care providers’ social skills lower than their parents did.²⁸

Conclusions

The results of this review suggest that family caregivers of children on HMV experience a variety of outcomes impacting their health and family functioning. Despite limitations to methods and design, as a collective the studies signal that family caregivers (FC) of children on HMV are at risk of deleterious effects consequent to extraordinary caregiving responsibilities. Studies also indicate that family caregivers appreciate the opportunity to have their child on HMV at home. Future studies are needed to better inform the development of targeted interventions, and inform health service delivery and related health care policy to support and sustain the valuable contributions of FC of children on HMV.

Recommendations for the published experiences and outcomes of family caregivers when a child is on long-term mechanical ventilation at home

1. *Family caregivers should receive an initial as well as ongoing psychosocial assessment related to the burden and costs (financial, emotional) associated with caring for a child on HMV. (Consensus)*
2. *Family caregivers should be assigned a 'key worker' or another accountable individual to provide professional care coordination and case management to ease the daily workload of caregiving. (Consensus)*
3. *Care of the child on HMV includes routine assessment of the health and well-being of family members including parents, siblings, and others involved in the child's care. If there are concerns regarding patient safety due to issues in the family, child protective services must be contacted. (Consensus)*
4. *As the use of HMV advances, family caregivers should receive personalized services using novel technologies, including high-fidelity simulation for training purposes that promote confidence, competence, enhanced problem solving, and optimal family function. (Consensus)*
5. *Technology-enabled video monitoring, or other technology-enabled modalities and support should be used for communication with the health care team to reduce family caregiver anxiety, provide symptom support, promote trouble-shooting of equipment challenges, and reduce unscheduled provider visits. (Consensus).*

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