Outcomes and Causes of Death in Children on Home Mechanical Ventilation via Tracheostomy: An Institutional and Literature Review

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Objective To describe outcomes and causes of death in children on chronic positive-pressure ventilation via tracheostomy.

Study design We conducted a retrospective observational cohort analysis of 228 children enrolled in an university-affiliated home mechanical ventilation (HMV) program over 22 years (990 person-years). Cumulative incidences of survival and liberation from HMV are presented. Time-to-events were compared by reason for chronic respiratory failure (CRF) and age and date of HMV initiation with Kaplan-Meier and Cox regression analyses. Circumstances of death are described.

Results Of our cohort, 47 of 228 children died, and 41 children were liberated from HMV. The 5-year cumulative incidences of survival and liberation were 80% and 24%, respectively. Being placed on HMV for chronic pulmonary disease was independently associated with liberation from HMV (hazard ratio, 7.38; 95% CI, 3.0-18.2; $P < .001$). Neither age nor reasons for CRF were associated with shortened survival. Progression of underlying condition accounted for only 34% of deaths; 49% of deaths were unexpected.

Conclusion Most children on HMV survive or were weaned off. However, a sizable number of children in our cohort died, and many deaths were unexpected and from causes not directly related to their primary reason for CRF. *(J Pediatr 2010;157:955-9)*.

Home mechanical ventilation (HMV) via tracheostomy is perhaps the most demanding and risky of technology dependencies. Since the 1960s, HMV programs in the United States, and later across the globe, have demonstrated the relative safety and efficacy of HMV for supporting children with chronic respiratory failure outside of intensive care settings.[1] Even with this life-sustaining support, these children have complex chronic conditions, often with associated co-morbidities, that put them at risk for critical illness and death.

Nineteen earlier institutional surveys have reported on the outcomes of 621 pediatric patients on chronic PPV via tracheostomy in 6 countries, with follow-up ranging from 4.5 to 25 years (**Table I**; available at www.jpeds.com).[2-20] Incidences of mortality in these studies ranged from 0 to 43%; incidences of liberation from PPV were 0 to 52%. These surveys had relatively small cohorts (6-101 patients), and most studies reported little on the circumstances of deaths in their cohorts. Some commentaries have suggested that the survival of these children is primarily influenced by the clinical course of their underlying disease.[3,12,21] In contrast, our anecdotal experience suggested that the deaths in our patients were from more varied causes and often not anticipated.

Therefore, we conducted a retrospective cohort analysis of our relatively large population of children and young adults on chronic positive-pressure ventilation (PPV) via tracheostomy to describe their outcomes and the circumstances of their deaths.

Methods

We performed a retrospective chart review of all patients on HMV who received full- or part-time chronic PPV via tracheostomy and were observed at Children's Hospital Los Angeles (CHLA) between November 1977 and April 2009. The CHLA Institutional Review Board approved this review. Data was extracted from paper charts, the Pediatric Intensive Care Unit database (Microsoft Access, Microsoft Corporation, Redmond, Washington), and the hospital database (Knowledge, Information, Decision Support, Cerner Corporation, Kansas City, Missouri). Limited, additional patient-specific information was obtained

CHS	Central hypoventilation syndromes
CHLA	Children's Hospital Los Angeles
CI	Confidence interval
CPD	Chronic pulmonary diseases
CRF	Chronic respiratory failure
HR	Hazard ratio
HMV	Home mechanical ventilation
PPV	Positive-pressure ventilation
VMW	Ventilatory muscle weakness

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Table III. Survival and weaning status of 228 home mechanical ventilation patients by reason for chronic respiratory failure

Cause of CRF	Total patients n (%)	Alive on HMV n (%)	Weaned		Died	
			n (%)	95% CI	n (%)	95% CI
CPD	120 (52.6)	62 (51.7)	35 (29.1)	21.2-38.2	23 (19.2)	12.6-27.4
VMW	62 (27.2)	45 (72.6)	4 (6.4)	1.8-15.7	13 (21)	11.7-33.2
CHS	46 (20.2)	33 (71.7)	2 (4.3)	0.5-14.8	11 (23.9)	12.6-38.8
Total	228	140 (61.4)	41 (18)	13.2-23.6	47 (20.6)	15.6-26.5

from the HMV program coordinator (S.K.) and the CHLA pediatric pulmonology faculty.

Data was collected on each patient's primary cause of chronic respiratory failure (CRF) and indication for HMV, sex, age at initiation of HMV, date of initiation, survival status, and, when applicable, age at death or liberation from HMV. CRF was defined as full- or part-time ventilator dependence for at least 1 month after repeated, failed attempts to wean from assisted ventilation in a person without superimposed acute respiratory disease. Causes of CRF were classified in 3 subgroups: chronic pulmonary diseases (CPD), ventilatory muscle weakness (VMW), and central hypoventilation syndromes (CHS). Examples of diagnoses for each subgroup can be found in Table II (available at www.jpeds.com). Patients with multi-factorial reasons for CRF were discussed with the program coordinator and the faculty member who knew the patient the best to decide their primary cause of CRF.

Survival status was determined to be alive on HMV, weaned off HMV, or deceased. For deceased subjects, we performed a more thorough review of their medical records to collect additional data on cause of death, place of death, and whether the death was expected. Expected death was defined as an attending physician documenting that the child was at risk for death outside of the hospital or, for inpatients, that the patient was at risk for death or that death was anticipated within one week. Documentation of anticipated death was not considered to be expected when the death followed acute, severe deterioration within 24 hours.

Patients who were >18 years of age at the time of HMV initiation or with insufficient information were excluded. Children who died after being liberated from HMV were analyzed in the weaned off HMV group.

Data are presented as medians and interquartile ranges (IQR) or as proportions and 95% CIs. Univariate analysis of patient characteristics and causes of death for different reasons for CRF were tested with the Fisher exact test and Kruskal-Wallis rank test, as appropriate. Kaplan-Meier survival and cumulative incidence of liberation from HMV curves are presented and compared with the log-rank test. The relationships among reason for CRF, age at HMV initiation, year of initiation, survival, and liberation from HMV were analyzed with Cox proportional hazards models. Statistical significance was determined with a P value of .05 and by constructing 95% CIs. Statistical analyses and graphs were performed with Stata software version 11 (StataCorp LP, College Station, Texas).

Results

Since 1977, the CHLA HMV program has cared for 388 children and young adults receiving full- or part-time chronic PPV via tracheostomy. One hundred forty-two patients (36%) were excluded because of insufficient information or loss to follow-up. The cause of CRF and the dates of HMV initiation, liberation, or death could not be confirmed in the medical record for 126 children (32%). Of these children, 73 (19%) were initiated on HMV before July 1987—the earliest date a study patient was started on HMV. Sixteen patients (4%) were lost to follow-up after 1987. Eighteen additional patients (5%) were excluded because they were initiated on HMV after 18 years of age. The remaining 228 patients (59%) comprised our study population and contributed 990 cumulative person-years of follow-up. The longest duration of follow-up was 212 months. Ninety-eight of 228 children (43%) were female.

Seventeen of 228 patients (7.5%) had a multi-factorial etiology of CRF—14 (6%) had both CPD and CHS diagnoses, and 3 (1.5%) had CPD and VMW diagnoses. For 12 patients, the non-CPD diagnosis was determined to be the primary cause of CRF.

The median age of initiation of HMV was 11 months (IQR, 6-41.5 months; range 1-210 months). When stratified by reason for CRF, children with CPD were initiated on HMV at significantly younger ages (median, 8 months) than children with VMW or CHS (median, 21.5 and 25.5 months, respectively; Kruskal-Wallis rank test, 17.1; $P = .0002$).

Table III details the survival status of our cohort with the incidences of all-cause mortality and liberation from HMV, with their 95% CIs, by reason for CRF. One hundred forty patients (61%) remain alive on HMV. For the entire cohort, the 5- and 10-year cumulative incidences of survival were 80% (95% CI, 73%-85%) and 63% (95% CI, 51%-73%), respectively.

Forty-one patients (18%) were liberated from HMV. Excluding patients who died, the 5- and 10-year incidences of liberation were 24% (95% CI, 18%-32%) and 29% (95% CI, 21-39%), respectively. Thirty-seven of 41 liberated patients (90%) were weaned within 5 years of initiation. All liberated patients were weaned to tracheostomy collar, and none required non-invasive ventilatory support. Two patients with