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Original article

Long-term home non-invasive positive pressure ventilation in children: Results from a single center in Japan

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Abstract

Background: Non-invasive positive pressure ventilation (NPPV) in children has recently increased worldwide and is used not only for neuromuscular diseases but for various other diseases. However, there have been few observational studies on long-term NPPV in children in Japan.

Methods: Based on medical records, we retrospectively evaluated patients aged ≤20 years who were initiated long-term NPPV at our hospital from January 2001 to December 2015.

Results: A total of 53 patients on long-term NPPV were identified; 38 (72%) had severe motor and intellectual disabilities (SMID). Compared to those with non-neuromuscular diseases, those with neuromuscular diseases had significantly more planned initiations and less frequent use of oxygen. Regarding patient outcome, 34 patients continued NPPV (64%), and there were three discontinues (6%), seven tracheostomies (13%), and nine deaths (17%). The continuation rate was high among those with neuromuscular disorders (15/19 cases, 79%) and that of tracheotomy was high in those with metabolic/degenerative diseases (3/9 cases, 33%). Ten patients transitioned to adult care, accounting for 29% of the 34 continuing patients.

Conclusion: This is the first observational study on long-term NPPV use in children in Japan that examined outcomes in patients with a range of disorders. The initiation situation, management, and outcomes differed between patients with neuromuscular and non-neuronal muscular diseases. Long-term use of NPPV is possible in many cases, including children with SMID, but can be challenging to continue in patients with progressive diseases such as metabolic/degenerative diseases. Careful discussions regarding the management of each patient are necessary.

Keywords: Long-term mechanical ventilation; Non-invasive positive pressure ventilation; Children; Severe motor and intellectual disabilities; Japan

1. Introduction

Use of non-invasive positive pressure ventilation (NPPV) in children has increased worldwide since the 1990s and has widely been used to manage acute and chronic respiratory failure [1–3]. The guidelines for Duchenne muscular dystrophy [4], spinal muscular atrophy (SMA), [5] and congenital myopathies [6] recommend NPPV for long-term respiratory management. NPPV is also used for various other conditions such as pulmonary diseases, abnormalities in the upper respiratory tract, and central apnea. In Europe and Canada, the use of tracheostomy positive pressure ventilation (TPPV) has decreased greatly with the increase of NPPV, which accounts for the majority of home

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mechanical ventilation used in children in recent years [7–10]. Long-term NPPV increase in infants under 1 year of age has also been reported [11]. In addition, NPPV has also increased as a form of palliative care in children with severe motor and intellectual disabilities (SMID). A national survey of NPPV in children with SMID in Japan reported that the number of NPPV cases was 248 in acute respiratory failure and 303 in chronic respiratory failure [12].

Although the long-term use of NPPV in children is increasing worldwide, there have been few detailed reports on this subject in Japan. Our hospital has administered long-term NPPV for various diseases since 2002 and has followed patients up over a long period. The present study was conducted to clarify the background, management, and outcomes of patients on long-term NPPV and to compare trends between their underlying disease classifications.

2. Subjects and methods

Based on medical records, we retrospectively evaluated patients aged 20 years or younger who were prescribed long-term NPPV by our hospital from January 2001 to December 2015 and followed up on these patients at our hospital. Our hospital is the only pediatric tertiary facility in Kanagawa Prefecture of Japan, which has a population of 9 million. We select a long-term respiratory management method (TPPV or NPPV) for the patients with chronic respiratory failure comprehensively according to their spontaneous respiration, respiratory tract, lungs, and degree of respiratory failure.

In this study, long-term NPPV was defined as NPPV management at home or a long-term facility continued for more than 3 months. Patients with continuous positive airway pressure therapy were excluded.

We investigated the year when NPPV was initiated, patient age at administration (in years), type of underlying disease(s), initiation situation, ventilation time, oxygen use (yes/no), use of mechanical insufflation-exsufflation (MI-E) use (yes/no), NPPV interface, and outcome. Underlying disease was classified as neuromuscular diseases, perinatal disorders, congenital anomalies (including chromosomal abnormalities, congenital infections, brain malformations, and multiple malformations), epileptic encephalopathies, metabolic/degenerative diseases, hypoxic ischemic encephalopathies, sequelae of acute diseases, and others.

In acute phase, NPPV is introduced as a management for acute respiratory failure due to airway infection or atelectasis or as a management after extubation. In chronic phase, NPPV introduction is considered when the patient has minimal pulse oximetry (SpO2) <92% or maximal transcutaneous carbon dioxide (PtcCO2) >50 mmHg chronically in a state where chronic hypoventilation is predicted. Initiation situation was classified as "acute setting" (initiated at the acute exacerbation of chronic respiratory failure and continued in the chronic phase) or "planned" (based on abnormal nocturnal gas exchange). Ventilation time was classified as "daytime and nocturnal" or "nocturnal" ventilation. If there was fluctuation in ventilation time, we adopted the time (i.e., all-day versus nocturnal) that was maintained stably for a longer period. NPPV interface was classified as nasal, nasobuccal, or unknown.

Patient outcome was classified as "continue," (continued use of NPPV at end of study) "discontinue" (NPPV became unnecessary due to improvement of respiratory condition), tracheostomy, or death. We evaluated CO_2 partial pressure in venous blood gas for adjustment of NPPV.

We also examined patients administered long-term TPPV during the same period, and compared the number of patients and types of underlying disease in the two patient groups (NPPV versus TPPV).

Mann-Whitney *U* test was used to compare the age at administration between patients with neuromuscular versus non-neuromuscular disease. Fisher's exact test was used to compare the initiation situation and oxygen use between patients with neuromuscular versus non-neuromuscular disease. Log-rank test was used to compare the duration of NPPV between patients with metabolic/degenerative and non-metabolic/degenerative disease. P-values <0.05 were considered statistically significant.

This research was approved by the ethics committee of Kanagawa Children's Medical Center (approval number: 1705-05).

3. Results

Fifty-three children commenced long-term NPPV during the study period, all of whom were cared for at home. All the patients had biphasic positive pressure ventilation. Seventy children received long-term TPPV during the same period. Use of NPPV gradually increased from 2003 and remained stable after 2009 (Fig. 1). In recent years, the number of patients receiving NPPV was comparable to or slightly less than the number receiving TPPV.

Congenital anomalies and hypoxic ischemic encephalopathy were the dominant underlying diseases in patients receiving TPPV, whereas neuromuscular disease accounted for the majority of underlying disease in patients receiving NPPV (Fig. 2). Detailed information on the underlying diseases in the study sample are shown in Table 1. Of these, 38 (72%) patients had SMID (including SMA type I and Fukuyama congenital muscular dystrophy). Although SMA type I does not originally complicate intellectual disability, we classified it as

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