

**Final Topic Refinement Document**  
**Home Mechanical Ventilators**  
**ID: PULT0717**  
**Agency for Healthcare Research and Quality**  
**Technology Assessment Program**  
**Mayo Clinic Evidence-based Practice Center**  
**16 October 2017**

**Preliminary Key Questions (KQs)**

- KQ1.** What are the patient characteristics and/or laboratory criteria and/or target level measurable improvements considered for the initiation and continuation of noninvasive positive pressure ventilation supplied by a Home Mechanical Ventilator (HMV), Bilevel Positive Airway Pressure device (BPAP), and Continuous Positive Airway Pressure device (CPAP) in the home through a noninvasive interface for the population of patients with chronic respiratory failure due to neuromuscular diseases, thoracic restrictive diseases, chronic obstructive pulmonary diseases (COPD), or other obstructive lung diseases (cystic fibrosis, bronchiectasis)?
- a. What are the patient characteristics and/or laboratory criteria and/or target level measurable improvements (e.g. reduction in hypercapnia) considered for the initiation and continuation of noninvasive positive pressure mechanical ventilation supplied by a HMV through a noninvasive interface in the home?
  - b. What are the patient characteristics and/or laboratory criteria and/or target level measurable improvements (e.g. reduction in hypercapnia) considered for the initiation and continuation of noninvasive positive pressure ventilation supplied as a BPAP through a noninvasive interface in the home?
  - c. What are the patient characteristics and/or laboratory criteria and/or target level measurable improvements (e.g. reduction in hypercapnia) considered for the initiation and continuation of noninvasive positive pressure ventilation supplied as a CPAP through a noninvasive interface in the home?

**KQ2.** In each of the above groups, what is the effect of HMV, a BPAP, or a CPAP use on patient outcomes, including mortality, hospitalization, admission/readmission to intensive care unit (ICU), need for intubation, outpatient visits, emergency room visits, disease exacerbations, quality of life (QoL), activities of daily living (ADL), dyspnea, sleep quality, exercise tolerance, and adverse events?

**KQ3.** What are the equipment parameters that are used in each of the above groups?

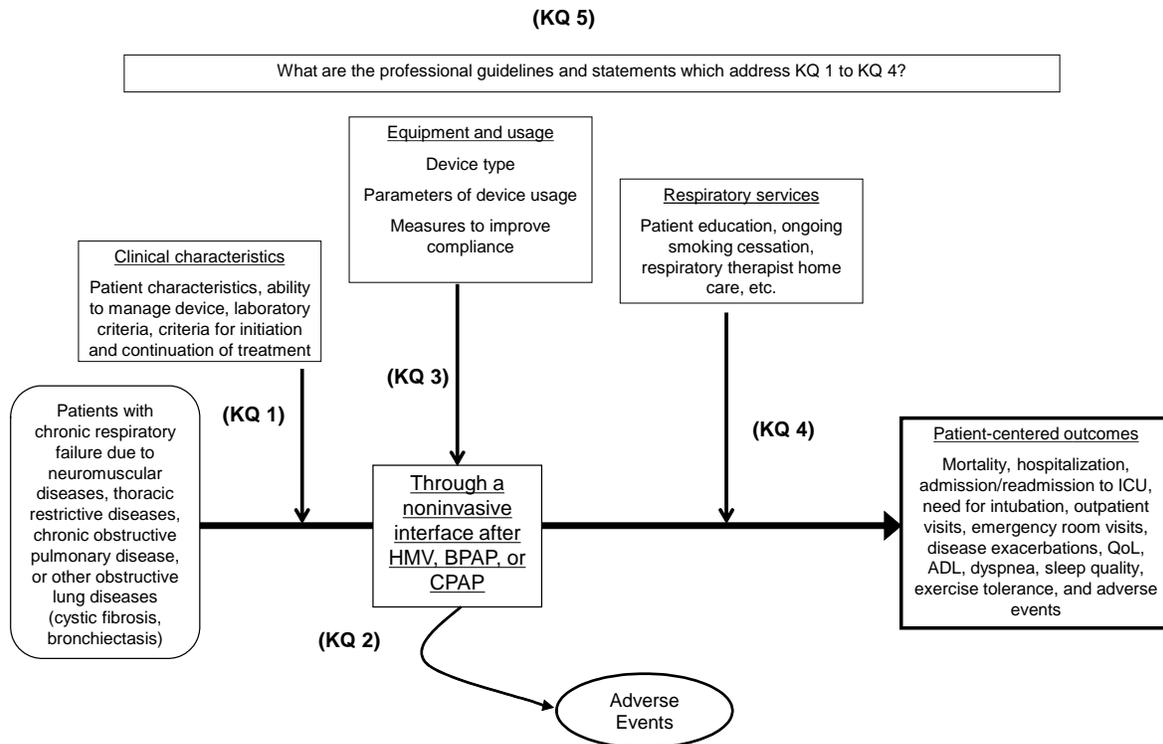
- a. What are the parameters of ventilator usage (e.g. mode as determined by trigger, control and cycling variables)?
- b. What are the equipment parameters that are necessary to achieve desired outcomes (e.g. flow capabilities, settings, etc.)?
- c. What are the parameters of prescribed patient usage (e.g. frequency of use, duration of use throughout the day, other)?
- d. In each of the above populations, what are the parameters of patient compliance with the prescribed usage of the equipment?

**KQ4.** What respiratory services, other than the technical support of the use of the prescribed equipment, are being provided to the above patients in the home (e.g. patient education, ongoing smoking cessation, respiratory therapist led home care)?

**KQ5.** What are the professional guidelines and statements which address KQ 1 to KQ 4?

## Preliminary Analytic Framework

Figure 1. Draft analytic framework for home mechanical ventilators



## Background

Chronic respiratory failure is a common condition with important morbidity and mortality and can require long-term home mechanical ventilation. Chronic respiratory failure is defined as the long-term inability to maintain oxygen and carbon dioxide levels within normal limits. Chronic respiratory failure may range from mild to severe and can be characterized as hypoxemic (inability to maintain a  $\text{PaO}_2 \geq 60$  mm Hg), hypercapnic (inability to maintain a  $\text{PaCO}_2 \leq 45$  mm Hg), or a combination of both. Many disease conditions may contribute to chronic respiratory failure including, but not limited to neuromuscular diseases, thoracic restrictive diseases (including thoracic cage abnormalities and morbid obesity), chronic obstructive pulmonary disease, and hypoventilation syndromes.<sup>1</sup> Such disease states and the extent of associated respiratory failure may be relatively stable over time or progressive in nature.

Mechanical ventilation is used to treat chronic respiratory failure. A mechanical ventilator is "a device capable of delivering pressurized gas (either through a secured artificial airway (tracheostomy) or through a mask or mouthpiece) in a manner that repeatedly supplies a physiological tidal volume to the lungs sufficient to improve or fully sustain respiration." Mechanical ventilator devices are broadly classified into two main categories: 1) home mechanical ventilators (HMV) and 2) bi-level positive airway pressure (BPAP) devices.<sup>1</sup> The United States Food and Drug Administration (FDA) has typically approved HMVs

using the “CBK” approval code and home BPAP machines using the “MNT” and “MNS” approval codes. In addition, some patients with chronic respiratory failure may benefit from continuous positive airway pressure (CPAP) devices.

While both HMs and BPAPs provide positive pressure ventilation, their technical features may vary considerably. Areas of device variability include: mode of ventilation (such as pressure targeted ventilation versus volume targeted ventilation), respiratory circuit (such as single-limb versus double-limb), presence of a flow sensor, user interface, monitoring capability (such as measured versus calculated inspired and expired tidal volumes), safety and alarm systems, internal battery life, and accessories. Devices also differ according to the interface provided (such as tracheostomy, mask, or mouthpiece), as well as level of oversight and servicing, and prescription of a second or backup machine.

If deemed to be feasible and safe, long term use of HMs and BPAPs is preferred in the home setting compared to other settings such as intensive care units (ICUs), ventilator weaning units, or long-term care hospitals. Home use has been associated with lower costs, greater independence, increased quality of life, decreased risk of hospital-acquired infections, and increased space for other acute care patients in acute care facilities.<sup>2-4</sup> The number of patients using long-term HMs as well as the money spent on HMs are growing.<sup>5</sup>

Failing to adequately treat chronic respiratory failure with the appropriate features of an appropriate mechanical ventilator device could potentially result in sudden or gradual hypoxemia and/or hypercarbia. These physiologic aberrations may result in several adverse outcomes that include, but are not limited to: death, respiratory arrest, need for emergency room evaluation, need for hospital admission, need for the intensive care unit admission, need for intubation, deterioration of health, hypersomnolence, and poor quality of life.<sup>1, 6</sup>

Selecting the most appropriate respiratory device to use for an individual patient is of highest importance. Determining the need for a HM versus BPAP versus CPAP is complex and relies on several important patient level and device level factors such as the underlying disease, interface required (a tight fitting removable mask versus a mouthpiece attachment), type of ventilatory support required, duration of ventilatory support needed per day, and required equipment characteristics

Currently, substantial variability exists regarding the usage, prescribing patterns, policies, and guidelines for HMs versus BPAPs versus CPAPs.<sup>7, 8</sup> This variability exists, even when accounting for variability in underlying disease processes and severity of chronic respiratory failure. While a number of guidelines exist regarding BPAPs and HMs in the home for different disease conditions, there is marked variability in the conclusions, recommendations, and evidence basis for such guidelines.<sup>9-12</sup> Many guidelines may address home BPAP usage and other guidelines may address HM usage, few guidelines address the intricacies of choosing one versus the other. With the current levels of practice variability, reimbursement variability, and unclear guidelines, there is a clear need to synthesize the highest quality evidence from the medical literature to clinically guide prescribing of HMs, BPAPs, and CPAPs.<sup>13</sup> Several challenges contribute to this variability.

1. There is considerable overlap regarding the technical features of HMs and BPAPs. While HMs traditionally provided volume targeted ventilation using an invasive tracheostomy interface and BPAPs provided pressure targeted ventilation using a mask interface, the FDA has approved HMs which can provide pressure targeted ventilation using a mask interface and BPAPs which can be used with an invasive tracheostomy interface. Some BPAPs and HMs can achieve the same flow delivery at essentially the same settings to the patient, and modes of ventilation can even be switched from BPAP type functions to HM type functions.<sup>14</sup>

2. There is considerable variability regarding the continuum of severity of chronic respiratory failure. Depending on the severity of illness, patients with chronic hypercapnic respiratory failure may require no ventilatory support, intermittent ventilatory support (during variable lengths of time at night or day or both), or continuous ventilatory support.
3. A significant newer body of literature (over the past 15 years) necessitates a reexamination of recommendations, guidelines, and policies regarding HMVs, BPAPs, and CPAPs. Such evidence synthesis can assist patients, family members, clinicians, professional societies, and policy makers regarding prescription and use of HMVs and BPAPs.

### **Population, Interventions, Comparators, Outcomes, Timings, and Settings (PICOTS) by Key Question (KQ)**

KQs 1-5:

#### **Population(s)**

- Adults older than 18 years with chronic respiratory failure due to neuromuscular diseases, thoracic restrictive diseases (including thoracic cage abnormalities and morbid obesity), chronic obstructive pulmonary diseases, or other obstructive lung diseases (cystic fibrosis, bronchiectasis)?

#### **Interventions**

- Home mechanical ventilators (FDA-approved only) with or without pertinent ancillary in-home services (e.g. respiratory therapy in the home; pharmacy reconciliation; smoking cessation, etc.)
- BPAP respiratory assist devices (FDA-approved only) w/ or w/o pertinent ancillary in-home services
- CPAP respiratory assist devices (FDA-approved only) w/ or w/o pertinent ancillary in-home services

#### **Comparators**

- Usual care (i.e. no mechanical ventilation/BPAP/CPAP)
- Different type of noninvasive mechanical ventilation
- Different modes of same equipment
- Other noninvasive ventilation

#### **Outcomes**

Patient-centered outcomes

- Mortality
- Hospitalization
- Admission/readmission to intensive care unit (ICU)
- Need for intubation
- Outpatient visits
- Emergency room visits
- Disease exacerbations
- Quality of life (QoL)
- Activities of daily living (ADL)
- Dyspnea
- Sleep quality
- Exercise tolerance
- Adverse events

#### **Timing**

- At least 1 month of treatment

#### **Setting**

- Home
- Assisted living residence

### Publication time

- From 1995

### Subgroup analysis

- Type of diseases
  - Neuromuscular diseases
  - Thoracic restrictive diseases
    - Thoracic cage abnormalities
    - Morbid obesity
  - COPD
  - Other obstructive lung diseases (cystic fibrosis, bronchiectasis)
- Length of treatment (1 month, 3 months, 6 months and longer)

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