

**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?

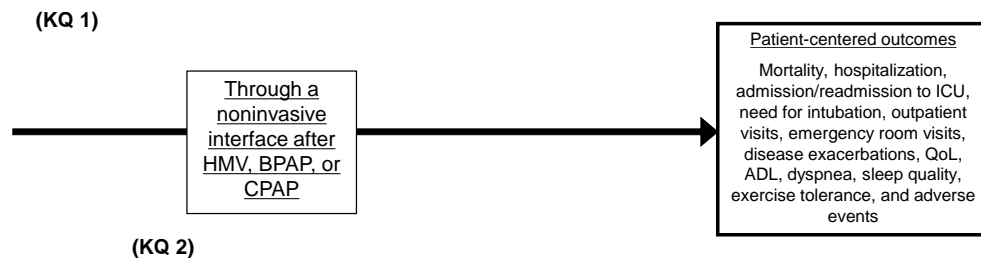
**KQ2.** In each of the above groups, what is the effect of HMV, a BPAP, or a CPAP use on patient outcomes, including mortality, hospitalization, admission/readmission to intensive care unit (ICU), need for intubation, outpatient visits, emergency room visits, disease exacerbations, quality of life (QoL), 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

## 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  $\text{PaO}_2$   $\bullet$   $\text{PP} + \text{J}$ ) hypercapnic (inability to maintain a  $\text{PaCO}_2$   $\text{''}$   $\text{PP} + \text{J}$   $\text{RU D FR PEL Qa n y l s e a G e R I E R W K}$ ) 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

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

## Publication time

- x From 1995

## Subgroup analysis

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

## References

1. King AC. Long-term home mechanical ventilation in the United States. *Respir Care*. 2012 Jun;57(6):921-30; discussion 30-2. doi: 10.4187/respcare.01741. PMID: 22663967.
2. Bach JR, Intintola P, Alba AS, et al. The ventilator-assisted individual. Cost analysis of institutionalization vs rehabilitation and in-home management. *Chest*. 1992 Jan;101(1):26-30. PMID: 1729079.
3. Marchese S, Lo Coco D, Lo Coco A. Outcome and attitudes toward home tracheostomy ventilation of consecutive patients: a 10-year experience. *Respir Med*. 2008 Mar;102(3):430-6. doi: 10.1016/j.rmed.2007.10.006. PMID: 18023334.
4. MacIntyre NR, Epstein SK, Carson S, et al. Management of patients requiring prolonged mechanical ventilation: report of a NAMDRRC consensus conference. *Chest*. 2005 Dec;128(6):3937-54. doi: 10.1378/chest.128.6.3937. PMID: 16354866.
5. Simonds AK. Home Mechanical Ventilation: An Overview. *Ann Am Thorac Soc*. 2016 Nov;13(11):2035-44. doi: 10.1513/AnnalsATS.201606-454FR. PMID: 27560387.
6. Murphy PB, Rehal S, Arbane G, et al. Effect of Home Noninvasive Ventilation With Oxygen Therapy vs Oxygen Therapy Alone on Hospital Readmission or Death After an Acute COPD Exacerbation: A Randomized Clinical Trial. *JAMA*. 2017 Jun 06;317(21):2177-86. doi: 10.1001/jama.2017.4451. PMID: 28528348.
7. MacIntyre E, Asadi L, McKim DA, et al. Clinical outcomes associated with home mechanical ventilation: A systematic review. *Can Respir J*. 2015 Sep 30 PMID: 26422402.

8. Lloyd-Owen SJ, Donaldson GC, Ambrosino N, et al. Patterns of home mechanical ventilation use in Europe: results from the Eurovent survey. *Eur Respir J*. 2005 Jun;25(6):1025-31. doi: 10.1183/09031936.05.00066704. PMID: 15929957.
9. Miller RG, Jackson CE, Kasarskis EJ, et al. Practice parameter update: the care of the patient with amyotrophic lateral sclerosis: drug, nutritional, and respiratory therapies (an evidence-based review): report of the Quality Standards Subcommittee of the American Academy of Neurology. *Neurology*. 2009 Oct 13;73(15):1218-26. doi: 10.1212/WNL.0b013e3181bc0141. PMID: 19822872.
10. McKim DA, Road J, Avendano M, et al. Home mechanical ventilation: a Canadian Thoracic Society clinical practice guideline. *Can Respir J*. 2011 Jul-Aug;18(4):197-215. PMID: 22059178.