Respiratory complications, management and treatments for neuromuscular disease in children.

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Abstract

PURPOSE OF REVIEW: To summarize current literature describing the respiratory complications of neuromuscular disease (NMD) and the effect of respiratory interventions and to explore new gene therapies for patients with NMD.

RECENT FINDINGS: Measurements of respiratory function focus on vital capacity and maximal inspiratory and expiratory pressure and show decline over time. Management of respiratory complications includes lung volume recruitment, mechanical insufflation-exsufflation, chest physiotherapy and assisted ventilation. Lung volume recruitment can slow the progression of lung restriction. New gene-specific therapies for Duchenne muscular dystrophy and spinal muscular atrophy have the potential to preserve respiratory function longitudinally. However, the long-term therapeutic benefit remains unknown.

SUMMARY: Although NMDs are heterogeneous, many lead to progressive muscle weakness that compromises the function of the respiratory system including upper airway tone, cough and secretion clearance and chest wall support. Respiratory therapies augment or support the normal function of these components of the respiratory system. From a respiratory perspective, the new mutation and gene-specific therapies for NMD are likely to confer long-term therapeutic benefit. More sensitive and standard tools to assess respiratory function longitudinally are needed to monitor respiratory complications in children with NMD, particularly the youngest patients.

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