

Prefazione

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The polio epidemic in the middle of the 20th century, had a major impact on the medical care of neuromuscular patients. Lasting more than ten years, this epidemic allowed significant progress particularly in respiratory care: NIV and secretion clearance techniques were established; long term tracheostomy techniques were mastered saving the lives of many; home care organizations were instituted which allowed patients to leave the hospital and re-enter their communities. These advances benefiting thousands of polio patients at the time however they were rarely used to treat other neuromuscular diseases until well into the 1980's. One reason to explain this is that unlike Polio patients, who often improved after the acute episode, patients with progressive and degenerative neuromuscular diseases were seen as having poor quality of life and therefore little was done to prolong their life.

Since the 1980's, developments in genetic research have identified the gene defect in many neuromuscular conditions and as a result provide some new hope for treatment or a cure of some conditions using gene therapy. These developments have had a significant change in the behavior of the medical community, patients and families and have led to a more enthusiastic attitude in the care of these patients. In addition, other therapeutic advances, such as the use of riluzol in ALS patients, have had a similar impact. With increasing potential for positive outcomes, families, patients and medical teams are placing more and more importance in preventing and treating complications such as scoliosis, respiratory failure, malnutrition, etc.

We can't ignore that during this same period of time there was a tremendous change in the public attitude toward patients with neuromuscular disease. Three factors contributed to this change. Telethons have taken place annually throughout the world increasing awareness of the problems facing neuromuscular patients and the potential solutions. Sensitizing the public to these issues has helped to raise a tremendous amount of money furthering research capabilities remarkably. Patient associations, which exist in most countries, (MDA, AFM, UILDM) and their lobbying activities, contributed to changing laws which protect the rights of the handicapped. These associations have also helped to standardize and improve overall care by organizing multidisciplinary clinics in various communities which focus specifically on the needs of the neuromuscular patients and improving the competence of the medical teams. These organizations are generally very strong and politically influential.

Another development since the 1980's which has had a significant impact on the natural history of a range of neuromuscular disorders, where respiratory insufficiency is the most common cause of premature mortality, is the revolution in ventilatory support. The application of ventilatory assistance, in many cases noninvasively, has extended survival considerably in many neuromuscular patients allowing some patients with nonprogressive pathologies to live to nearly normal life expectancy, while improving their quality of life.

Studies looking at quality of life in very dependent neuromuscular patients have shown that in all domains other than physical ability, patients score themselves as having a good quality of life equal to the non-handicapped. Ventilator dependent patients scored no differently than those patients without ventilation. When asked the same questions as their patients, medical *caregivers* significantly underestimated their patient's quality of life. Understanding this disparity has helped to change physicians attitudes.

This review "Evaluation and treatment of neuromuscular disease and amyotrophic lateral sclerosis" provides the current state of the art regarding the care of neuromuscular patients. You will find outstanding information in each chapter so I will only mention a few:

- The second chapter covers the multidisciplinary care approach. This type of organization around the patient is essential due to the multiple and varied consequences of their disease. Incorporating a Care coordinator helps to synthesize the results from each medical discipline and to assure communication. The paramedical staff continues to play a major role in this type of organization as they are often the people who spend the most time with the patient and family, providing therapeutic treatment and education. Finally, this multidisciplinary organization can help to assure a positive transition between pediatric and adult services.
- The fifth chapter covers acute situations describing their causes and appropriate treatment. While reading this important chapter, it is important to keep in mind that hospitalization is difficult for anyone but often catastrophic for a physically dependent patient. The lack of expertise in the general management of physically dependent patients and their equipment, coupled with the fact that their usual caregivers can rarely accompany them, makes any hospital a particularly difficult environment for the neuromuscular patient. It is therefore extremely important to help the medical team be aware of the special needs of these patients. Finally, much effort is required to anticipate, prevent, and prepare for these types of situations. Early patient and family information, regular evaluations, precise action plans in case of complications can help to prevent and manage complications and hospitalizations.
- The last chapter is related to managing end of life situations. This is a subject that has been considered "tabou" in many communities and it is especially the case in our Latin societies. This subject can no longer be ignored due to the availability of information (internet), medical progress and advanced technology. It is therefore important to read this chapter attentively as there is excellent information on how to help patients, their families and the medical team manage end of life situations.

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In particular you will find information about the importance in providing information to patients early, involving the family physician in the decision making, guaranteeing patient dignity, respecting patient wills and providing access to palliative care if needed.

Major progress in the care of neuromuscular disease patients has occurred in the last few years and this review provides an excellent state of the art. Enjoy it!