The ATS Guidelines in the respiratory management of Duchenne muscular dystrophy.

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The 2004 American Thoracic Society publication of the guidelines for respiratory care in Duchenne muscular dystrophy (1) can be viewed as the result of a “perfect storm” of factors leading to its publication. New, non-invasive technologies had become available for respiratory management of the patient with DMD. Respiratory care protocols for prevention of pulmonary morbidity (2) as outlined by Bach, et al, had gained wide acceptance among respiratory specialists, while among the non-pulmonary physicians, these therapies were not being prescribed. The major respiratory complications of DMD (inadequate cough and inadequate ventilation) now had specific, non-invasive therapies. Yet the major insurers (both private and Medicaid insurers) were refusing to pay for these therapies, citing a lack of efficacy data performed in the usual randomized, prospective manner. The mechanical insufflator-exsufflator (MI-E), which had been shown quite efficacious in small physiological and retrospective studies, was still deemed “experimental therapy” by most payers. In the 1990’s, many clinicians charged with the care of DMD patients viewed the disease as fatal and untreatable. Therapeutic nihilism (e.g., the “take them home and love them” approach) was common, and many families were not offered even moderately aggressive approaches towards maintenance of respiratory health.

The 90’s also saw the rise of the parental advocacy movement, in parallel with the growth of the internet, instantly linking geographically separate parents. The parent advocacy organization, Parent Project Muscular Dystrophy was founded in 1994.

The final factor was the rise of what has been referred to as the “guidelines movement.” This refers to an increasing trend among medical specialties to create practice parameters and care documents based on careful review of the medical literature. “Evidence-based medicine” is an offshoot of this movement, with an emphasis on empiric data in medical decision-making.

As a result of the above factors, a number of the authors of the ATS statement found themselves frustrated by an inability to deliver appropriate care to their patients. The major impediment was, of course, insurance coverage. Meanwhile, parents around the country became aware of a newer standard of care for Duchenne patients through the internet and through involvement with parent groups like the Parent Project. This became the incentive to create a consensus
statement: If the insurance companies were denying coverage citing a lack of literature, we would have to create it ourselves in the form of an expert consensus panel.

The ATS panel was formed after an interest group was created on the internet and by invitation based on publication and expertise. So as not to have solely pulmonologists, one neurologist and one nurse with expertise in Duchenne care were also invited to round out the panel. The initial meeting took place in 2001 at the annual American Thoracic Society meeting, with follow up meetings in 2002 and 2003. Teleconference meetings were held to create a forum for discussion and to form consensus on all aspects of care. The final manuscript was reviewed by 6 peer reviewers and by the entire Board of Directors of the American Thoracic Society and published in August 2004.

One might presume that such a document, in which non-invasive respiratory management, mechanically assisted coughing, multi-disciplinary care, and a strong support of the family in all decisions would have opened many doors for patients. In many areas this is the case. For example, at present most state Medicaid programs cover the mechanical insufflator-exsufflator. Yet the majority of private payers still do not cover this device, even in the face of a published expert panel consensus report supporting its use.

Experience and training also dictate practice patterns. Since most clinicians involved in the care of Duchenne patients have not been trained in the non-invasive respiratory management of these patients, such therapies are not even offered in some communities. At present the Muscular Dystrophy Association has not established minimal standards of care for their supported clinics (although they are clearly moving in this direction).

Worldwide, the same issues exist. The national health programs of Australia, for example, do not support mechanically assisted cough in Duchenne MD, and private funds are often used to get these devices into communities. Often a single device must serve a single community, despite the epidemic nature of respiratory infections. Many nations do not offer this technology to the patients, nor can families afford to obtain such care.

In summary, much work remains to aid the patients with Duchenne in the United States and worldwide. There remains a strong role for parental advocacy especially in the realm of improving insurance coverage of life-saving technologies that prevent pulmonary morbidity. There remains a shortage of specialists trained in the respiratory management of Duchenne MD nationwide and worldwide. Although for many the situation has greatly improved since the
2004 consensus statement, much work remains to help those who remain in crisis.

References:
