Caring for Neuromuscularly Weak Patient

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QuickTime™ and a YUV420 codec decompressor are needed to see this picture.
Introduction

• Primary morbidity & mortality of NMD is respiratory
• Cost of complications >> cost of prevention
• New technologies & new paradigms have changed how we manage pts with NMD
• I will focus basic respiratory care of NMD, non-invasive care of SMA
Part I: Duchenne MD
Guillaume Benjamin Amand Duchenne de Boulogne (1806-1875)

Duchenne’s drawing of pt with muscular dystrophy

Duchenne with patient
Duchenne’s experimentation with electrical stimulation

http://chem.ch.huji.ac.il/~eugeniik/history/duchenne.html
Duchenne MD

- The best studied and most common pediatric NMD
- Incidence 1:3,000 males (similar to CF)
- Easily predictable course
- Majority of morbidity is respiratory
- Cause of death 80% respiratory causes
  - (Entirely preventable)
Survival in DMD: Room for Improvement.

The following data from 1983-1997 are from a poster presentation from the CDC:

Hereditary Progressive Muscular Dystrophy Deaths, N=13,095.

<table>
<thead>
<tr>
<th>Age Group</th>
<th>Count</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>0-12</td>
<td>62.7%</td>
<td>Male</td>
</tr>
<tr>
<td>13-29</td>
<td>95.6%</td>
<td>Male</td>
</tr>
<tr>
<td>30-99</td>
<td>59.4%</td>
<td>Female</td>
</tr>
</tbody>
</table>
Figure 3: Regional Differences in Median Age at Death

<table>
<thead>
<tr>
<th>Region</th>
<th>Median</th>
<th>Mean</th>
<th>N</th>
</tr>
</thead>
<tbody>
<tr>
<td>Northeast</td>
<td>20</td>
<td>20.539</td>
<td>945</td>
</tr>
<tr>
<td>Midwest</td>
<td>20</td>
<td>20.188</td>
<td>1334</td>
</tr>
<tr>
<td>South</td>
<td>19</td>
<td>19.889</td>
<td>1700</td>
</tr>
<tr>
<td>West</td>
<td>20</td>
<td>20.251</td>
<td>878</td>
</tr>
</tbody>
</table>

p<0.001

p=0.021
Evidence-based medicine?

• Most literature is anecdotal and retrospective
• Most RTC studies have involved steroids
• As a result there has been a delay in approval of many life-saving therapies
• Clinical equipose does not exist today:
  – Unethical to withhold effective therapies to study them
Many issues: therapeutic nihilism, lack of training...

• Crosses all borders (US\textsuperscript{1}, Canada\textsuperscript{2}, Switzerland\textsuperscript{3})
• Few physicians prepared to care for adults with DMD\textsuperscript{4}
• QOL studies reflect overall satisfaction (except for “love life”)\textsuperscript{4, 5}

\textsuperscript{5}Raphael JC, \textit{Revue Neurologique}. 158(4):453-60, 2002 Apr
Stages of respiratory function in Duchenne MD

1. Normal (age 0-10)
   - Vaccinate, educate
2. Inadequate cough (age 10-15)
3. Inadequate night time ventilation (age 15-20)
4. Inadequate daytime ventilation (>age 17)
   - These ages vary greatly!
Stage 1: Normal Resp Function

- Rule of thumb: ambulatory pt does not require assistance with cough or breathing
- Aside from usual risks of anesthesia no special risks
- Pulmonary function testing in pts over age 6
- Immunization (influenza & s. pneumo.)
Stage 2: Impaired cough

- 2 forms of airway clearance:
  - Mucociliary escalator (impaired in CF, PCD, smokers, etc)
  - Cough clearance (impaired in NMD but also by tracheostomy)
Stage 2: Inadequate cough

- Often asymptomatic until a respiratory tract infection
- Easily predicted with PFT’s and/or measurement of “peak cough flow”
- Peak cough flow <160 L/min associated with failure to extubate
- PEFR/PCF <270 L/m is indication for assisting cough


Assisting cough: Manual Assisted Cough

- Abdominal thrust or thoracic squeeze after a maximal insufflation with AMBU bag or vent breath
  - Do this on an empty stomach
  - Scoliosis and contractures of thoracic wall limits effectiveness of this technique
Manually assisting cough
Fortunately for Sparky, Zeke knew the famous "Rex maneuver."
Mechanically assisted cough

- Much preferred to direct tracheal suctioning in tracheostomized patients (more effective, too)
- Can be used via mask, mouthpiece, or tracheostomy
- Achieves peak cough flows in the effective range even in severely weak patients
- Prophylactic use prevents atelectasis, supports chest wall compliance
Mechanically Assisting cough:

- Mechanical Insufflation-Exsufflation (MI-E)
- Respironics “CoughAssist” device
- Very effective in clearing secretions
- Takes getting used to!
- Pressures should be at or above -30 cm H2O (expiratory)
Physical Methods Simulating Mechanisms of the Human Cough

ALVAN L. BARACH, GUSTAV J. BECK, HYLAN A. BICKERMAN, and H. EUGENE SEANOR, with the technical assistance of WILLIAM SMITH. From the Department of Medicine, College of Physicians and Surgeons, Columbia University, and the Presbyterian Hospital, and Goldwater Memorial Hospital, Columbia Division, and Willard Parker Hospital, New York City

The need for development of physical methods of eliminating retained bronchial secretions is especially evident in patients with poliomyelitis, in whom the absence of an effective cough is largely responsible for the fatal pulmonary complications. Inadequate removal of mucoid and mucopurulent material from the lungs takes place in other clinical entities, such as myasthenia gravis, bronchial asthma, pulmonary emphysema, bronchiectasis, lung abscess, blocked tuberculous cavities and pulmonary atelectasis due to a variety of causes, including respiratory depression induced by anesthetic or sedative drugs. The purpose of this paper is to report studies made on two mechanical techniques which were developed with the intention of simulating certain characteristics of an effective cough in human subjects.

Observation of patients with bronchopulmonary disease revealed two types of coughing: 1) The individual takes a deep breath, the glottis is closed, the abdominal muscles are contracted and the levator and depressor muscles of the ribs are held rigid; the intrapulmonary pressure is thereby increased until the glottis is suddenly opened. A high volume flow rate of air is present at the beginning of expiration. The bronchi, relatively dilated and lengthened at full inspiration, abruptly narrow and shorten at the end of the cough. 2) In conditions such as bronchial asthma and bronchiectasis, in which mucoid or mucopurulent material may be lodged in small bronchi, a paroxysm of coughing frequently takes place. After an inspiration of variable depth a progressive decrease in chest volume is observed. The mucus may not be expectorated until the last of the series of coughs takes place. The chest wall is successively contracted during expiration rather than held rigid at the end of a full inspiration. During paroxysmal coughing the decrease in the lumen of the bronchi may be marked, with at times almost complete approximation of the bronchial walls, as has been observed during bronchoscopy and recently demonstrated roentgenologically (1). Expulsion of bronchial secretions may presumably be facilitated when air passes through narrowed bronchi at high velocity and moves the occluding mucus ahead of it. However, impairment of effective expectoration may also take place in patients with bronchial asthma and pulmonary emphysema as a result of excessive intrapulmonary pressure producing collapse of the bronchi or trachea (3). Since patients who are unable to produce an effective cough frequently suffer

Received for publication August 23, 1953.

1 These studies were supported by grants from the National Foundation of Infantile Paraly
The first mechanical exsufflator, 1952

Barach, 1952
Physical Methods Simulating Mechanisms of the Human Cough: Elimination of Radiopaque Material From the Bronchi of Dogs

HYLAN A. BICKERMAN, GUSTAV J. BRUCK, CHARLES GORDON AND ALVAN L. BARACH, with the technical assistance of SYLVIA ITRIN. From the Department of Medicine, College of Physicians and Surgeons, Columbia University, and the Presbyterian Hospital, and the Goldwater Memorial Hospital, Columbia Division, New York City

In addition to its primary function of gaseous exchange the pulmonary system is remarkably effective in its capacity for self-cleansing. The factors involved in the protection of the alveoli against foreign material include ciliary action, peristaltic-like contraction of the bronchial muscle, mucus formation, phagocytosis and cough. Of these, cough is apparently the most effective mechanism whereby the lung rids itself of excessive secretion and inhaled foreign matter. When the cough reflex is seriously impaired through paralysis or weakness of the respiratory musculature, bronchial secretions accumulate and obstructive dyspnea ensues, accompanied by physiopathologic changes in the lungs.

In the preceding paper a description was presented of two physical methods of producing high volume expiratory flow rates. The bronchial dynamics and the elimination of radiopaque material from the tracheobronchial tree of anesthetized dogs are presented in this paper.

Methods

In each experiment two anesthetized dogs weighing 6 to 12 kg. were used. Each dog was bronchoscooped and under direct visualization a catheter was placed in the right main bronchus. In one series of animals 15 cc. of mucopurulent expectoration from patients with bronchial asthma and bronchiectasis were introduced through the catheter. In a second group of animals radiopaque material was used for comparative bronchography. Considerable difficulty was encountered in finding a suitable contrast medium for these studies. The ideal substance should be nontoxic, resemble sputum in viscosity and be adequately radiopaque. Disodium appeared to be toxic and Lipiodol did not simulate the physical characteristics of mucoid or mucopurulent expectoration. A Thorotrust-mucin mixture provided excellent visualization. Four to six cc. of 24 per cent Thorotrast in aqueous suspension thickened with gastric mucin was instilled into the bronchial tree of each dog, and bronchograms were taken in the postero-anterior and right lateral positions. X-rays of the treated and control animals were repeated at the end of the experimental period, which was generally 2 hours.

Fifty cubic centimeters of a barium sulfate suspension were introduced into the...
Bickerman, 1952

- Showed exsufflation effective for clearing airways in canine model.
Emerson Cough Assist

“In-exsufflator”
MI-E -- Indications

- Neuromuscular weakness
- Peak cough flow <270 L/min
- Maximum expiratory pressure <60 cm H2O
- History of difficulty clearing secretions
- NOT indicated for CF
CoughAssist Video
WARNING!

• Treatment of low oxygen saturation with supplemental oxygen can be dangerous
• Will further suppress respiratory drive
• Can lead to respiratory failure and death
• Low saturation means increased airway clearance, need for increased ventilation
• Pulse oximeter very helpful
Pulse oximetry and $O_2$

- Development of mucus plugs can be insidious and silent
- Patients with NMD should have pulse oximeters available in the home
- $SaO2 < 95\%$ is indication for aggressive use of MI-E
- Oxygen is NOT used to treat hypoventilation!
1950’s: Early respiratory care experience in Polio shapes later care in MD

1952 Emerson infant “iron lung”
Stage 3: Nocturnal Hypoventilation

- Morning headache
- Increasing # of nocturnal awakenings
- Nightmares of smothering or drowning
- Poor quality of sleep leads to daytime sleepiness, poor school performance, etc
- Nocturnal hypoxemia noted on O/N oximetry or on polysomnography
Note that FVC<30% in DMD correlates to ventilatory failure (but not in SMA)

Selected cohort FVC% and funct score pred of need for MV in DMD not SMA II (youngest pt 9
Management of nocturnal hypoventilation

- Avoid tracheostomy, avoid suppl. O₂
- BiPAP or other positive pressure ventilator
- Avoid CPAP
  - Increases WOB w/o increasing ventilation
- Getting a comfortable mask fit is essential.
- Titrate to normal pCO₂ in sleep
- Patient should awaken feeling refreshed
Effect of NIV on diurnal arterial blood gas tensions

Table 1. – Diagnostic categories, mean age at starting noninvasive positive pressure ventilation (NIPPV) and diurnal arterial oxygen ($P_{a,O_2}$) and carbon dioxide ($P_{a,CO_2}$) tensions before and after NIPPV by diagnostic categories

<table>
<thead>
<tr>
<th>Diagnosis</th>
<th>Age yrs</th>
<th>$P_{a,O_2}$ kPa</th>
<th>$P_{a,CO_2}$ kPa</th>
<th>$P_{a,O_2}$ kPa</th>
<th>$P_{a,CO_2}$ kPa</th>
</tr>
</thead>
<tbody>
<tr>
<td>CMD</td>
<td>11.6±3.7</td>
<td>8.2±1.2</td>
<td>6.6±1.28</td>
<td>10.65±2.2</td>
<td>5.97±0.9</td>
</tr>
<tr>
<td>SMA</td>
<td>5.7±4.2</td>
<td>7.9±1.5</td>
<td>6.1±0.85</td>
<td>11.9±1.1</td>
<td>5.4±1.2</td>
</tr>
<tr>
<td>CM</td>
<td>7.3±3.3</td>
<td>8.2±0.6</td>
<td>7.73±1.25</td>
<td>11.3±1.15</td>
<td>6.00±0.9</td>
</tr>
<tr>
<td>DMD</td>
<td>13.9±1.5</td>
<td>9.3±2.3</td>
<td>6.33±1.13</td>
<td>12.17±1.46</td>
<td>5.75±0.41</td>
</tr>
<tr>
<td>Misc</td>
<td>9.4±4.8</td>
<td>9.4±2.3</td>
<td>7.6±1.37</td>
<td>9.33±1.87</td>
<td>6.33±1.1</td>
</tr>
<tr>
<td>Overall</td>
<td>9.4±1.8</td>
<td>8.5±1.8</td>
<td>7.0±1.6</td>
<td>10.9±1.7</td>
<td>5.9±0.8</td>
</tr>
</tbody>
</table>

Bear in mind:
Many noses -- many interfaces

Dibujo de George Cruikshank (1792-1878)
Various interfaces used for noninvasive positive pressure ventilation. Standard nasal masks in different sizes (Respironics, Inc.) (upper left), oronasal mask with very soft silicone seal (Resmed, Inc.) (upper right), nasal "pillows" (ADAM Circuit, Puritan Bennett, Inc.) (lower left) and mouthpiece attached to lipseal (lower right).
Stage 4: 24 hour ventilation dependence

- In years past, this was indication for trach
- No longer has to be the case!
- Most patients can be managed non-invasively with mouthpiece ventilation
- Newer, lightweight ventilators facilitate portability and remaining in school or at work
Non-invasive Positive Pressure Ventilation (NIPPV)

• First described by Alexander in 1979

• Non-invasive use of PPV best described by Bach
Bach’s protocol

• Assisted cough
• Non-invasive ventilation
• Use of pulse oximetry to determine need for increased MI-E
• Change to 24 hour ventilatory assistance at times of URI
Stress is on *anticipation* of respiratory care

NON-INVASIVE management also emphasized

Access to *specialty care* important:
- Pulmonologist
- Nutritionist
- Cardiologist
- Orthopedist
- Physical, speech, and occupational therapists; psychiatry, pastoral care as needed
Gaining World-wide Acceptance…

American Thoracic Society Documents

Respiratory Care of the Patient with Duchenne Muscular Dystrophy
ATS Consensus Statement

This official statement of the American Thoracic Society was approved by the ATS Board of Directors March 2004.

Documenti dell’American Thoracic Society

Assistenza Respiratoria del paziente con Distrofia Muscolare Duchenne

Il documento ufficiale dell’ATS è stato approvato dal consiglio dei direttori dell’ATS nel marzo 2004.

Italian translation courtesy of PPMD Italy
Part II: SMA
A joint project of FSMA and SMA Foundation

International group met Palo Alto, CA (Stanford U) in May 2006

Consensus Statement for Standard of Care in Spinal Muscular Atrophy 2007; 22; 1027 J Child Neurol
Themes from SMA conference

- Families deserve to have options presented (without prejudice)
- Non-invasive support is preferred to invasive support
- Early use of NIPPV is beneficial
- Aggressive nutritional support
Chest wall & lung development in congenital neuromuscular disease

Typical bell-shaped chest in SMA with pectus

Many thanks to Anita Simonds for these images
Chest wall development following NIV in SMA

Pt at 6 months

Pt at 18 months after initiation of NIPPV
Survival in Type I SMA without ventilatory support

SMA I: To support or not to support?

- Scenario/questionnaire based survey
  - sent to US intensivists, neurologists and physiatrists (response rate 34-57%)
- Intensivists less likely than physiatrists to recommend support
- Intensivists also less likely to offer respiratory support in acute circumstances
- High proportion of patients offered ‘comfort care’ only
- Wide variation in practice between specialties and recommendations given to families
Bach, 2000

- Protocol-based care: 11 pts with SMA-I
- IPAP >14; EPAP 3 cm H2O
- All managed non-invasively
- 48 intubations/28 hospitalizations
  - (9/11 pts)
- 3 pts nocturnal BiPAP only
- 2 pts 24-BiPAP dependent
- 1 pt tracheotomized (died)
- 1 pt lost to follow up
- Survivals mean 35 months
  - Range 12-81 months
- All pectus lesions resolved
Outcome of children with neuromuscular disease admitted to PICU
Yates K et al Arch Dis Child 2004;89:170-5

- Paediatric Intensive Care Unit, Westmead, Sydney
- Retrospective chart review n= 28 children, 69 admissions,
- 47% SMA II or myopathy
- 16 (57%) had > 1 admission
- Median PICU stay = 4 days (range 0.5-42)
- 23% unplanned admissions resulted in initiation of respiratory support
- Severity of functional impairment was NOT associated with longer stay or higher mortality score
- 10 children died, 4 in PICU
- Most recovered and were discharged without need for invasive ventilation, but long term NIV use was common
To sum up
Joey with SMA1, BiPAP in sleep, doing well
Kevin, age 17, in ventilatory failure, 24 hour vent-dependent...
Patrick, age 26, graduating from Pitt Law
Attorneys and Professionals

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M. Patrick Daniels is a staff attorney in the Pittsburgh office of Buchanan Ingersoll PC. He is a member of the firm’s Commercial Litigation Section.

Before joining Buchanan Ingersoll, Patrick gained experience through several legal externships while attending law school. In 2001, he worked with the Disabilities Law Project, where he drafted a brochure designed for parents of disabled pre-school children to inform them of their rights regarding daycare services. He also drafted a memorandum discussing the use of "testers" in enforcing fair housing laws.

During 2002, Patrick worked in the U.S. Attorney's Office, Western District of Pennsylvania, in the criminal division of the narcotics/violent crime section. There, he drafted memoranda discussing whether specific facts warranted a particular charge, the application of the "knock-and-announce" rule to federal agents and the use of expert testimony in narcotics cases.

In 2003, Patrick added to his experience by working with the Honorable Chief Justice Ralph Cappy in the Pennsylvania Supreme Court. He drafted allocutus reports and researched material for a speech given by the chief justice titled "Candor to the Court."

Patrick earned his J.D. degree from the University of Pittsburgh School of Law in 2004. He received an Outstanding Achievement Award, as well as the CALI Excellence for the Future Award. He also participated in the Murray S. Love Trial Moot Court. Patrick earned his B.A. degree in political science from Wright State.
• Goal is to reduce unnecessarily high peri- and intraoperative mortality in DMD
Thank you!
That’s all for now