Improving Respiratory Care for Patients with MD:
A consensus project

Jonathan D. Finder MD
Associate Professor of Pediatrics
University of Pittsburgh School of Medicine
Muscular dystrophy is a respiratory disease!

- OK, so it’s really a muscle disease
- Yet most of the mortality is respiratory:
  - Pneumonia
  - Respiratory failure
  - Difficulty clearing secretions
80% of DMD mortality is respiratory.

- Unnecessary!
- Predictable
- Treatable
- Preventable
Why are MD patients different?

• Very few doctors are trained in caring for the respiratory needs of patients with muscular weakness
• Most MD “specialists” are neurologists and not experts in breathing disorders
• MD pts can “fall between the cracks”
• Parents must advocate for the best of care!
Why are respiratory specialists important in NMD mgmt?

- Most serious illness is respiratory!
- New technologies & new ways of managing patients
- Specialized testing required

Kevin, age 14
The 2 basic concepts:

- Support breathing
- Support airway clearance
Standards of care?

• MDA has never set minimal standards of care
• PPMD advocated for and supported a consensus project (a small investment of $5K) in 2001
• Result: ATS statement, August 2004

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.
Why a consensus statement?

- MD is relatively rare (1:3,000 boys)
- Hard to do large controlled studies
  - Like in asthma, for example
- The medical literature is largely “anecdotal”
- Withholding effective treatment to “study” it is UNETHICAL
- HUGE CHASM between what is OUT there and what patients RECEIVE
Why a consensus statement?

• Insurance companies do not listen well to individual physicians but DO listen to expert consensus statements.
• Documents like this give parents a BATTERING RAM to force insurance companies to provide proper care!
• Children are dying unnecessarily and prematurely!
• It’s a CRISIS!
Survival: 1990’s, USA, Varies by Region (it should not!)
PPMD-Sponsored Respiratory Consensus Conference

- Official conference of American Thoracic Society
- Held Atlanta May, 2002 & 2003
- 11 pulmonary physicians, 1 neurologist and 1 nurse from all parts of the USA
- Publication date: August 15, 2004
- American Journal of Respiratory Care and Critical Care Medicine (AJRCCM)
ATS 2004 Consensus Statement

• Stress is on *anticipation* of respiratory care
• NON-INVASIVE management also emphasized
• Access to *specialty care* important:
  – Pulmonologist
  – Nutritional support
  – Cardiologist
  – Orthopedist
  – Physical, speech, and occupational therapists; psychiatry, pastoral care as needed
Consensus Statement Outline: I

• Evaluation
  – Respiratory testing
  – Sleep
  – Scoliosis screening
  – Nutrition
  – Cardiac
Consensus Outline II

• Management
  – Airway clearance
  – Non-invasive breathing support
  – Scoliosis
  – (Corticosteroids)
  – End of life care
4 respiratory stages in MD

1. Initially: normal respiratory function
   - Birth to age 10 or so
2. Normal breathing, but weak cough
   - Early teens
3. Normal breathing during daytime, but inadequate breathing asleep
   - Mid teens
4. Inadequate breathing awake and asleep
   - Mid-late teens
Stage 1: Normal

- Physical therapy directed to *chest wall*
- A good idea to get **immunized** against influenza annually and to receive Pneumovax once
- Annual screening **Pulmonary function tests** > age 6
- Identify the pulmonary specialist for your child
Stage II: Inadequate COUGH

- May not know until you get a cold
- *Easily predictable* with pulmonary function testing
- Main risk is PNEUMONIA
- You will notice a lot of difficulty clearing secretions with a cold
Mechanics of coughing

1. Deep breath (get air behind mucus)
2. Close the voice box
3. Contract the abdominal muscles
4. Open the airway
5. Air rushes out at a high velocity, carrying with it mucus and debris
Why MD patients get pneumonia

- Inability to take a deep breath
- Inability to generate good expiratory flow rate to expel mucus
  - Retention of secretions
  - Bacterial growth
  - Leading to pneumonia
Weakened cough: What can YOU do?

(A LOT, it turns out!)

1. **Manually assisted cough**: abdominal thrust following deep (or assisted breath with ambu bag or other device).
   - Fair at best

2. **Mechanically assisted cough**: The Emerson Cough Assist™ (In-Exsufflator) -- A fantastic device, a gift to the MD community.
Manually assisting cough
The key: PREVENTION!

- **Assisted cough** (Cough Assist™) with colds
  - ABSOLUTELY CRITICAL! The best way of preventing pneumonia, most reliable means...
- **Immunizations**
- **Percussion and drainage**
  - Only useful if you can get those secretions OUT!
- **Nebulized medications** when prescribed
- **Prompt medical attention** and resp. support with colds/lower resp. infections
The Vest?

- High frequency chest wall compression (HFCWC)
- Used in cystic fibrosis
- *Likely* of value in HOSPITAL but not at home
- no literature yet to justify its use as *policy*
- Expensive! $17K
Role of Pulse oximetry

- All patients with NMD requiring assisted cough should have pulse oximeter
- Saturation < 95% = aggressive airway clearance (CoughAssist)
- DO NOT USE O₂ as a substitute for cough or weak breathing muscles
Stage III: Inadequate breathing in sleep

- Symptoms may be subtle
  - fatigue, lack of restful sleep, morning headache, nightmares, increased # awakenings

- Easy to detect with overnight, in-home study with pulse oximeter or sleep study in hospital

- Most common solution is BiPAP
  - Bilevel Positive Airway Pressure
BiPAP

- BiPAP™ can support breathing in sleep
- Nasal mask or face mask
- Uncomfortable to use continuously, so not a good option for 24 hr support
Getting the interface to match

Severely limited options in pts < 1 yr
Payer limitations
Stage IV: Inadequate breathing all the time

• This stage often occurs following a severe infection, like a pneumonia.
• Respiratory insufficiency can be shown with PFT’s.
• No longer is tracheostomy mandated at this stage.
Non-invasive breathing support

Portable ventilator with a mouthpiece attached (like a microphone) to wheelchair has been successful for daytime or 24hr support

– Pulmonetic LTV 950 = 22 lbs
– LP10 (easier to get) = 50 lbs
– Tremendous improvement in energy level and quality of life
Invasive support of breathing

- Consider when a person cannot breathe sufficiently and cannot use mouthpiece vent
- Tracheostomy -- only in rare cases.
- Can be attached to wheelchair -- goal is mobility!
  - Lightweight vent is key to this.
- Can facilitate suctioning and can be used with CoughAssist
Managing colds

• Begin freq. use of CoughAssist at first sign
• Call your doctor
• Keep an eye on saturation with pulse oximeter
  – Avoid using oxygen - treats the symptom and not the disease
• Watch for signs of worsening shortness of breath. Infection itself can cause further muscle weakness.
In summary:

• Key to good health is anticipating respiratory needs:
• Don’t wait until a crisis occurs!
• Take a preventive approach
• Get the technology you need to stay away from the hospital!
• Identify a respiratory care professional interested in the care of MD patients
Your homework assignment:

• Identify a respiratory specialist for your son
• Begin annual PFT’s age 6
• *Demand* CoughAssist device when PEFR < 270 or difficulty with colds
• Keep a sharp eye out for sleep problems
  – Watch out for folks who would prescribe O2 alone
• Close contact with doc with colds
Patrick, age 26, graduating from Pitt Law, 2004
Admitted to Bar 2004, gainfully employed as an attorney!