Respiratory Therapy in SMA

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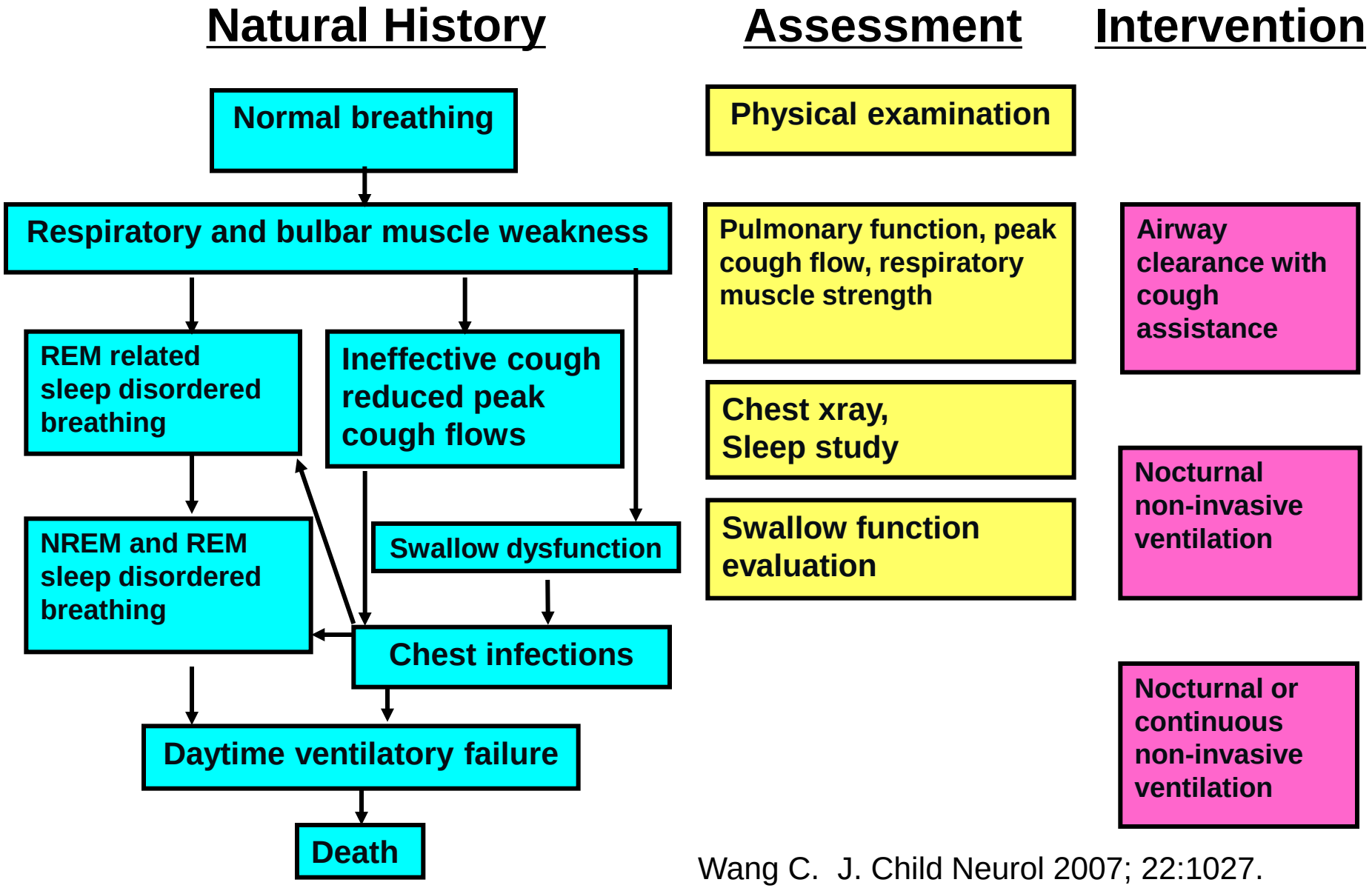
Objectives

- Describe our standard approach to the patient with SMA Type 1 as well as those with Types 2/3
- Discuss the modalities available for airway clearance in SMA
- Present the supporting data for these therapies
- Think outside of 

Results of Respiratory Muscle Weakness in SMA

1. Difficulty coughing
2. Small shallow breaths during sleep: hypoventilation
3. Chest wall and lung underdevelopment
4. Recurrent infections that contribute to muscle weakness

SMA Pulmonary Natural History



Wang C. J. Child Neurol 2007; 22:1027.

Standard Approach at Our Center

- Diagnosis made and patient seen in SMA clinic
- Options discussed with family including: palliative, non-invasive and invasive care
- If choice is to pursue treatment, we recommend a gastric tube placement with Nissen fundoplication
- Recovery from this procedure is done in PICU with extubation directly to non-invasive ventilation
- Mechanical cough assist is utilized Q2-Q4-BID
- BIPAP is weaned to use overnight as tolerated (full vent support overnight)
- Discharge planning completed for home

Fundoplication/GT Placement

Early laparoscopic fundoplication and gastrostomy is safe and is associated with improved nutritional status. A trend toward fewer significant long-term aspiration-related events was seen after fundoplication.

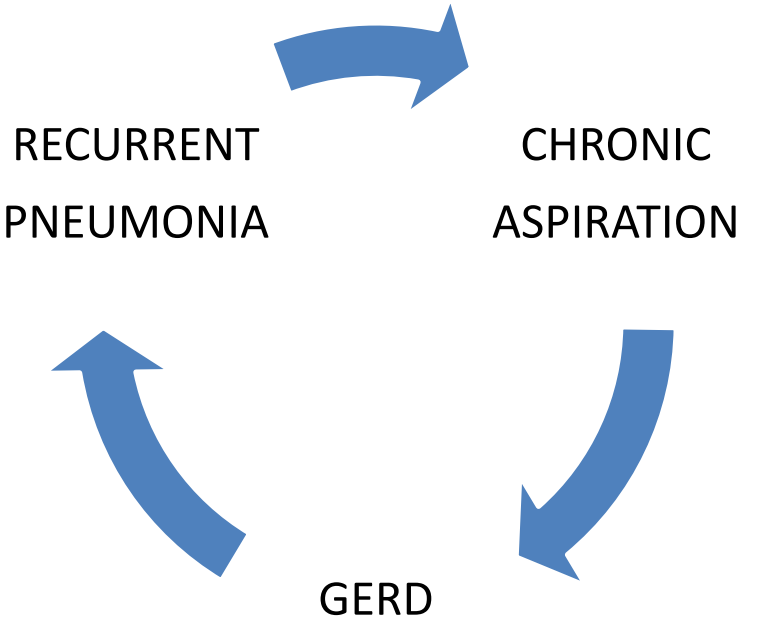
Durkin, E. J Ped Surg 2008 43,2031-2037

The SMA Social Media World

- Opinionated
- Demanding
- Advocates
- Strongly against any palliative approach
- Disseminates many unproven therapies, especially with nutrition
- Sometimes a support – Sometimes a barrier
- Some families do have a hard time navigating
- Makes care very difficult at times

Mucociliary Clearance

EARLY IN DISEASE PROCESS PATIENTS HAVE NORMAL CLEARANCE



IN SOME PATIENTS, THIS MAY CAUSE A CHRONIC INFLAMMATORY STATE AFFECTING CLEARANCE

Mucociliary Clearance

- Manual: percussion and postural drainage 
- Assisted: **Effort Independent**
    - High frequency chest therapy (VEST)
    - Intrapulmonary pressure ventilation (IPV)

Effort Dependent

- Airway oscillation devices (Acapella)



Mucociliary Clearance

IPV	VEST
• Resolve atelectasis • Less antibiotics • Less school absenteeism • Less hospitalizations • Safe • Not appropriate for infants or children	• Well studied in CF, ALS and CP • Less hospitalizations • Improved pulmonary function • Good adherence • Safe, tolerated in children

- No studies in SMA … therapies borrowed from other diseases and other forms of neuromuscular disease (where benefit was shown)
- Not much consensus in the standard of care
- Are we wrong to use it? Some patients demand we order it.

Finder, J: Ped Res Rev 2010;11:31-34
Deakins,K: Resp Care 2001;47:1162-1167
Reardon,CC: Arch Ped Adolesc Med 2005;159:526-431

Cough Augmentation (M-IE)

Manual/Mechanical Insufflation-Exsufflation

Manual I-E

- Stacked breaths to full inflation
- Abdominal thrust or thoracic squeeze as patient coughs

Mechanical I-E

- Positive pressure inspiration
- Negative pressure at airway opening as patient coughs

- Cochrane Review 2013 “There is currently insufficient evidence for or against the use of M-IE in people with neuromuscular disease.”
- Consensus statement for DMD recommends a cough flow of **<270 lpm** as the place to discuss cough augmentation
- Has been shown to increase vital capacity in neuromuscular disorders
- <u>In SMA Type 1, we really can’t measure this so when do we start?</u> At diagnosis….

Vital Cough vs Cough Assist

Features	Vital Cough	Automatic CA-3000	Cough Assist T70
Positive Pressure Range	10–50 cm H_2O	5–60 cm H_2O	+70 cm H_2O
Negative Pressure Range	–15–50 cm H_2O	–5–60 cm H_2O	–70 cm H_2O
Inhale Flow Settings	Low, Medium, High	Low, High	Low, Medium, High
Mode of Operation	Automatic and Manual	Automatic and Manual	Automatic and Manual
Inhalation, Exhalation, and Pause Times	0–5 Seconds	0–5 Seconds (not available in Manual mode)	0–5 Seconds (not available in Manual mode)
Lock Feature	Yes	No	Yes
Remain Resting/Pause Pressure	0–15 cm H_2O (adjustable)	No	No
Visualization/Setting	LCD 24 Bit Color Display/with Touch Screen	Mechanical Gauge/Knob	Screen with Buttons and Toggle Switches
Weight	6.8 kg (15 lbs)	11 kg (24 lbs)	• 3.8 kg (8.4 lbs) without Detachable Battery • 4.3 kg (9.4 lbs) with Detachable Battery Installed
Unit Dimensions	15.7" L x 10" W x 7.4" H	11.5" H x 11" W X 16.5" D	11.5" W x 9.1" H x 9.5" D
Remote	Foot Pedal	No	Foot Pedal

Other issues: direct ordering vs DME use

Chart provided by HillRom

Cough Augmentation

- This therapy gets at the root cause of the pulmonary issues: ineffective cough
- Settings are not standard: studies show 15-40 cmH2O as a place to start, some people are recommending higher (60)
- When to start is not standardized: SMA 1 ASAP vs SMA 2/3 similar to DMD? Our center starts right away.
- How often do we recommend use: BID, TID, PRN
- Insurance coverage/Homecare support

Finder, J: Ped Res Rev 2010;11:31-34
Bach, J: Chest 1993:104:1553-1562
Panich, HB: Resp Care 2006;51:885-893
Finder, J: Respir Crit Care Med 2004:170;456-465
Stehling, F: Chronic Res Dse 2014;12:31-35
Schroth, M: Pediatrics 2009;123:S245-S249

Pharmacotherapy

- Albuterol
- Ipratropium bromide (Atrovent)
- Pulmozyme
- N-acetylcystine (Mucomyst)
- Normal saline
- Hypertonic saline
- Inhaled steroids (in patients with airway hyperresponsiveness)

The use of aerosolized agents to change sputum physical properties or improved airway clearance cannot be recommended for patients with NMD or weakness due to insufficient evidence.

Strickland J Sl: Resp Care 2015 60:1071-1077

The first "powered" or pressurized inhaler was invented in France by Sales-Girons in 1858

Prevention

Recommended in patients with neuromuscular disease annually (no SMA studies)

3-10X higher rate of hospitalizations for RSV in high risk infants could justify use in first two seasons (no SMA studies)

Recommended in patients with neuromuscular disease

(no SMA studies)

AAP Committee on Infectious Disease and Bronchiolitis Guidelines 2104;134:e620-e638
Fauroux, B. Ped Res Rev 2009;10 Supp 1:21-22
Resch, B Op Micro J 2014; 8:71-77
Resch, B Ped Res Rev 2009;10:148-153