WHAT DO WE WANT?

- Optimize?
- Strive?
- Foster?
- Ensure?
- Partner?

We want to enjoy the time we have.
Management of children with spinal muscular atrophy type 1 in Australia

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N = 35

Total parenteral nutrition, gastrostomy and fundoplication were not provided for any children

“Conclusion: There appears to be a consistent approach in the management of children with SMA 1 in Australia, which can be characterised as ‘actively managed dying’.”
letting nature take its course (NT);

traceostomy and invasive mechanical ventilation (TV);

Continuous noninvasive respiratory muscle aid (NRA),
ENTEROSTOMY TUBE PLACEMENT IN CHILDREN WITH SPINAL MUSCULAR ATROPHY TYPE 1

K. SY, BSc, S. MAHANT, MD, N. TABACK, PhD, J. VAJSA, MD, P. G. CHAITT, MBCH, and J. N. FRIEDMAN, MBCHB

Objective To determine the major complication rate in the first 30 days after enterostomy tube insertion in infants with spinal muscular atrophy (SMA) type 1.

Study design A retrospective case review of all children with SMA type 1 who had a gastrostomy or gastrojejunostomy tube placed by the image-guided technique at the Hospital for Sick Children from 1994–2004. Major complications were classified as peritonitis, aspiration pneumonia, respiratory failure, nonelective admission to the pediatric intensive care unit, and death.

Results Twelve children were identified as having SMA type 1 with an enterostomy tube insertion. The median age at tube insertion was 6.1 months (range 2.2 to 15.8 months). Major complications in the first 30 days after the procedure included aspiration pneumonia (5/12 patients [41.6%]), respiratory failure requiring admission to the pediatric intensive care unit (4/12 [33%]), and death (2/12 [16.7%]). Children with development of aspiration pneumonia were significantly older at time of tube insertion ($P < .05$) than those with no aspiration.

Conclusions Major complications including death are seen in children with SMA type 1 in the first 30 days after enterostomy tube insertion. *(J Pediatr 2006;149:837-9)*

N = 12

5/12 (42%) “Aspiration pneumonia”

2/12 (17%) “Death”
Treatment of Type I Spinal Muscular Atrophy With Noninvasive Ventilation and Gastrostomy Feeding

David J. Birnkrant, MD*, John F. Pope, MD*, James E. Martin, BS, RRT†,
Anthony H. Repucci, MD*, and Robert M. Eiben, MD*


“We considered these patients poor candidates for Nissen fundoplication because of the potential risks of the procedure, especially in patients with respiratory compromise.”

BOTTOM LINE: Uniformly bad outcomes in Gastrostomy without Fundoplasty (but with ”maxium medical therapy”)
N = 7

“The average number of pneumonias and hospitalizations was reduced by 50%”

Actually it was better: The number of pneumonias in seven patients in 12 months (or less) PREop was 17.

In the 12 month POSTop, there were only 4 episodes.
Early laparoscopic fundoplication and gastrostomy in infants with spinal muscular atrophy type I

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“N= 12...
All patients were extubated immediately postoperatively.

There were no significant complications.

The number of respiratory-related hospitalizations in the cohort decreased by almost 50% in the ensuing 12 months after surgery...”
WHAT DO WE BELIEVE SO FAR?

• Interventions extend life
• Nutrition support is needed
• G-tube only is associated with a very high (40%) probability of aspiration within 30 days
• Fundoplication has low morbidity in these patients—but it’s surgery.
• Fundoplication appears to powerfully reduce relative risk of aspiration and pneumonia
SMA-1
What is the moral course?
Nothing
NG Feeds
Gastrostomy
Laparoscopic Nissen + G
Exclude other options:

- NG is “no operation”;
- Lap gastrostomy alone incurs all the same operative risks
All models are wrong; some are useful.

\[ P(D|X_{1..i}) = \prod_{1}^{i} P(X_i) \]

\[ R = \frac{\sum P(D | \sim F')}{\sum P(D | F)} \]
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RR of Death | No Fundo 2.59

Over a range of plausible assumptions...

...SMA-1 pts 2X—3X more likely to die without fundo!
Small decreases in aspiration risk bring big risk reductions for early death.
No Nissen

Laparoscopic Nissen + G

- Very Low Operative Morbidity
- Less Aspiration Risk
- Fewer Hospitalizations
- "Better" Death

No Anesthesia Risk

No Operative $Cost
“CONSIDER FUNDOPPLICATION FOR ALL SMA-1 BABIES…”

- **RR of death** in 12 mos *without* fundo is ~2-3x higher
- Lap Nissen/G appears to have **low cost** ($, pain, time, complications)
- We think this operation **extends life, reduces hospitalizations**, and **changes the likely form of death** (i.e. decreases sepsis+ICU)