Chest and Spine Deformity of the Young Patient:
The Bottom Line

RM Campbell,
Division of Orthopaedics, CHOP
Director
CHOP Center for Thoracic Insufficiency Syndrome
Disclosures

- Grant Support
  - National Organization of Rare Disorders (NORD)
- NORD Medical Advisory Committee member
- Spinal Consultant to the FDA
- Advocate for inventors/companies trying to develop safe and effective devices for children
“Growth Friendly” Instrumentation: We’ve got a lot more to use now

- VEPTR- approved 2004
- Growing Rods-used past 30 years, now cleared
- MAGEC-recently cleared
- SHILLA-recently cleared

Outcomes
- Curve control
- Spinal growth
- Complications
- Await pulmonary outcomes
- Await long term mortality
Bottom Line

• Will the child live a normal lifespan?
• Will the quality of life be acceptable long term?

• Is your treatment outcome better than natural history?
Natural History of Scoliosis

High Death Rate develops later in life well after we stop following them.

3 X Normal

2 X Normal

Normal

Figure 2. Cumulated number of observed deaths in patients with infantile, juvenile and adolescent scoliosis, and expected deaths.
Long Term Treatment History of Scoliosis

A long term survival study is needed
Long term VEPTR treatment of Jarcho-Levin Syndrome

Karlin, J. , Campbell, et al., JBJS 2014

- 10 spondylocostal dysostosis (SCD)
  - Age surgery 3 yr, f/u 8 yrs
- 19 spondylothoracic dysplasia (STD)
  - Age surgery 4yr, f/u 6.2 yr
Two surgical approaches

- SCD
- STD
• T spine height 99.3 mm
  (62% nl) preop
  f/u 141mm (65% nl)
  (24% nl, natural hx,
   Ramirez, JBJS 2007)
• L spine height 67.2
  (69% nl) preop
  f/u 85.9mm (70% nl)
Pulmonary: STD

Respiratory Rate

• Pre-op  31 bpm
• Post-op  24 bpm

Full Ventilator Dependence

– 3 pts pre-op
– 1 pt at f/u

FVC

• Earliest post op 52% predicted
• Latest f/u  39.4% predicted

(Natural Hx, < 30% predicted, Ramirez, et al., JBJS 2007)

CTIS
Long Term f/u of VEPTR treatment for Jeune Syndrome

O’Brien, A, Campbell, et al., JPO, in press

- 24 pts
  - 2 lost to f/u
  - 17 > 2yr f/u

- Avg age at surgery 23 months
- f/u 8.4 years (2.3–15.6 yrs)
- Infection rate 4.6%/procedure
C1 cervical stenosis in 16%
Jeune’s Syndrome

C-1

Chest
The spine is normal in height in Jeune syndrome
Scoliosis in 41% of pts preop. The remainder developed curves with treatment.
Respiratory

- 7 full time ventilator dependent pre-op
  - Only 2 at f/u
- 3 on room air pre-op
  - 6 on room air f/u
- RR decreased from 35 bpm to 24 bpm
- FVC % predicted 34% at first test, 27% at last f/u
VEPTR Treatment of Jeune Syndrome

Mortality

• Natural history 70-80% mortality

• VEPTR treated ( n= 22 )
  – 4 pts had early demise
    • 2 with multi-system disease
  – 68 % are survivors

f/u 8.4 years (2.3–15.6 y)
Can we cure Thoracic Insufficiency Syndrome?

no
What is the reality of long term FVC?

FVC (nl = 80-100% predicted)

Normals

TIS

X

Treatment?

Birth 20 yrs 40 yrs 60 yrs

Jeune FVC 34% → 27%
STD FVC 52% → 39.4%

-300 ml - 700 ml

SMA II/III: natural history and fusion FVC

Preop

Post Fusion

Fig. 1 Pre-operative percentage of predicted forced vital capacity (FVC) versus age in years.

Fig. 2 Post-operative percentage of predicted forced vital capacity (FVC) versus age in years.

Duchenne MD and Scoliosis

Untreated,
FVC↓ 5% /yr

-Roberto, et al, SPINE, 2011
Duchenne MD and Scoliosis

Treated with Spine fusion, FVC \( \downarrow \) 4%/yr

- Roberto, et al., SPINE, 2011
Duchenne MD and Scoliosis

Immediate 11% ↓ FVC after fusion

- Roberto, et, SPINE, 2011
Is Vital capacity the only thing to consider?

- Forced Vital Capacity
  - The “sprint” of respiration

- Tidal Volume: Breathing at rest
Like testing total hip replacement outcome by a 100 yard dash
To treat a disease, you first must understand it.
Having instrumentation:
Great feeling!

Really understanding the disease you are trying to treat:

Priceless...
What really is a Rib Hump?
True Patho-anatomy of the Rib Hump in Adolescent Idiopathic Scoliosis

James Peters BS ¹, Sriram Balasubramanian PhD ¹
¹ School of Biomedical Engineering, Science and Health Systems
Drexel University, Philadelphia, PA

Lucy Robinson PhD ²
² School of Public Health, Drexel University, Philadelphia, PA

Robert M. Campbell Jr. MD³
³ Division of Orthopaedic Surgery, CHOP

FUNDED BY THE SCOLIOSIS RESEARCH SOCIETY
2013 NEW INVESTIGATOR RESEARCH GRANT
Results and Conclusions

N=13 AIS subjects (10F, 3M)
Mean age: 14.15 ± 1.41 years
Cobb Angle: 54.38±16.16 degrees

• No significant bilateral differences (in rib pairs) were observed in rib length, rib enclosed area and apparent rib curvature
CTIS
WHOLE RIB CAGE

SUBJECT #1

SUBJECT #2

SUBJECT #3

RED – RIGHT RIB
BLUE – LEFT RIB

@ T7
@ T5
@ T3
@ T9
The rib hump is the collapsing parasol deformity, not angulated ribs.
Complications
What degree of device complications are tolerable?

The good done outweighs the bad
We have not defined in growth modulation surgery an important concept.
## Surgical Site Infection Rates

1347 procedures

### Growing Instrumentation constructs

<table>
<thead>
<tr>
<th>Category</th>
<th>Insertion</th>
<th>Revision/Lengthening</th>
</tr>
</thead>
<tbody>
<tr>
<td>Syndromic</td>
<td>28%</td>
<td>8-10%</td>
</tr>
<tr>
<td>NM</td>
<td>10%</td>
<td>6.8%</td>
</tr>
<tr>
<td>Congenital</td>
<td>7%</td>
<td>2.8%</td>
</tr>
<tr>
<td>Infantile/Juvenile Idiopathic</td>
<td>0%</td>
<td>3.6-29%</td>
</tr>
</tbody>
</table>

(mostly growing rods, few VEPTRs included)
These are all different diseases
Must compare
treatment complications
to the
complications of natural history, not normal
Device Issues: Ventriculo-Peritoneal Shunts for Hydrocephalus

• Infection occurs in 3%–27% of cases after shunt insertion.
V-P shunts

- Failure rates 70% in the 1st year after surgery and 5% annually thereafter
- Shunt failure rate has not changed significantly since 1960
- Shunt-related hospital admissions account for $1.4–2.0 billion in hospital charges yearly
  - Patwardhan Rv et al. Neurosurgery 56:, 2005
Despite complications, it’s better than natural history
The Timing of surgical treatment?
Delay to avoid complications of long term treatment?
Avg. Predicted Normal Vital Capacity at follow-up: VEPTR tx’d Fused ribs and congenital scoliosis

< 2 yr at VEPTR surgery

>2yr at VEPTR surgery

>2 yr with hx fusion

-Campbell, Smith et al., JBJS 2004
Intervene before thoracic function loss is irreversible.
“EVIDENCE BASED MEDICINE”

- There’s a lot of weak and misleading "evidence" out there
- Consider a "Principles Based" medicine approach
  - Keep it logical and simple, honestly consider all issues, be transparent
The Principles Based Approach

Whatever it takes to have:
1. Biggest
2. Most Symmetrical
3. Most Functional
   Thorax
by skeletal maturity
Some final comments

• Be critical about new things
  – Everything looks good that first 5 years

• Be especially critical about your own ideas
  – Being your own worst critic helps you anticipate problems and find early solutions

• Everything fails one way or another
  – Design safe failure modes
The Bottom Line

• We need to learn more about the diseases we are treating

• We need long term follow-up
  – Mortality
  – Pulmonary outcomes, PFTs and clinical

• We need realistic treatment goals
  – Quality of life
  – Extension of life
Thank You!