

The Children's Hospital of Philadelphia®

Hope lives here.

**Center
for
Thoracic Insufficiency
Syndrome**

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

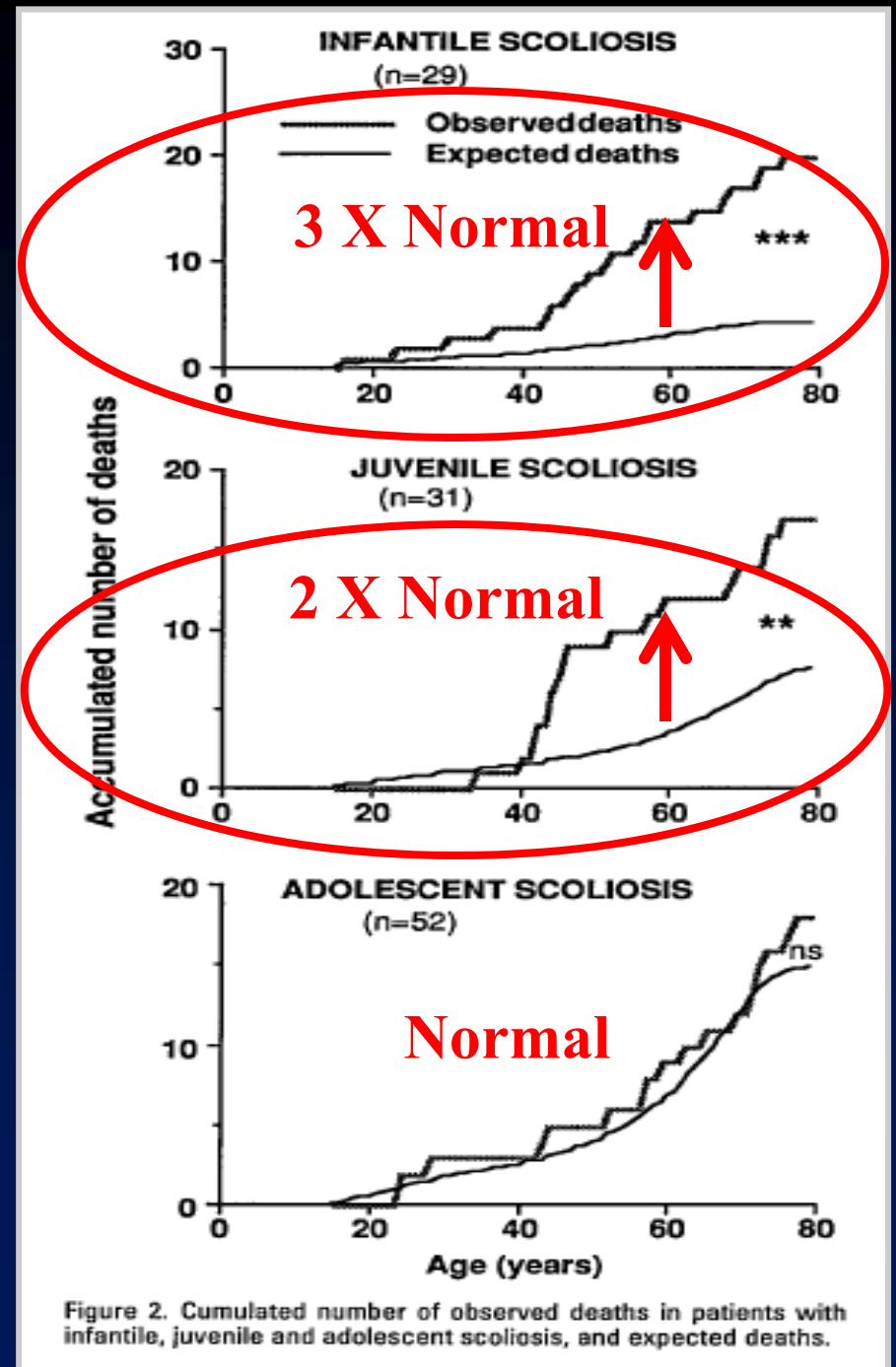
- Curve control
- Spinal growth
- Complications
- Await pulmonary outcomes
- Await long term mortality

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



Long Term Treatment History of Scoliosis

A long term
survival study
is needed

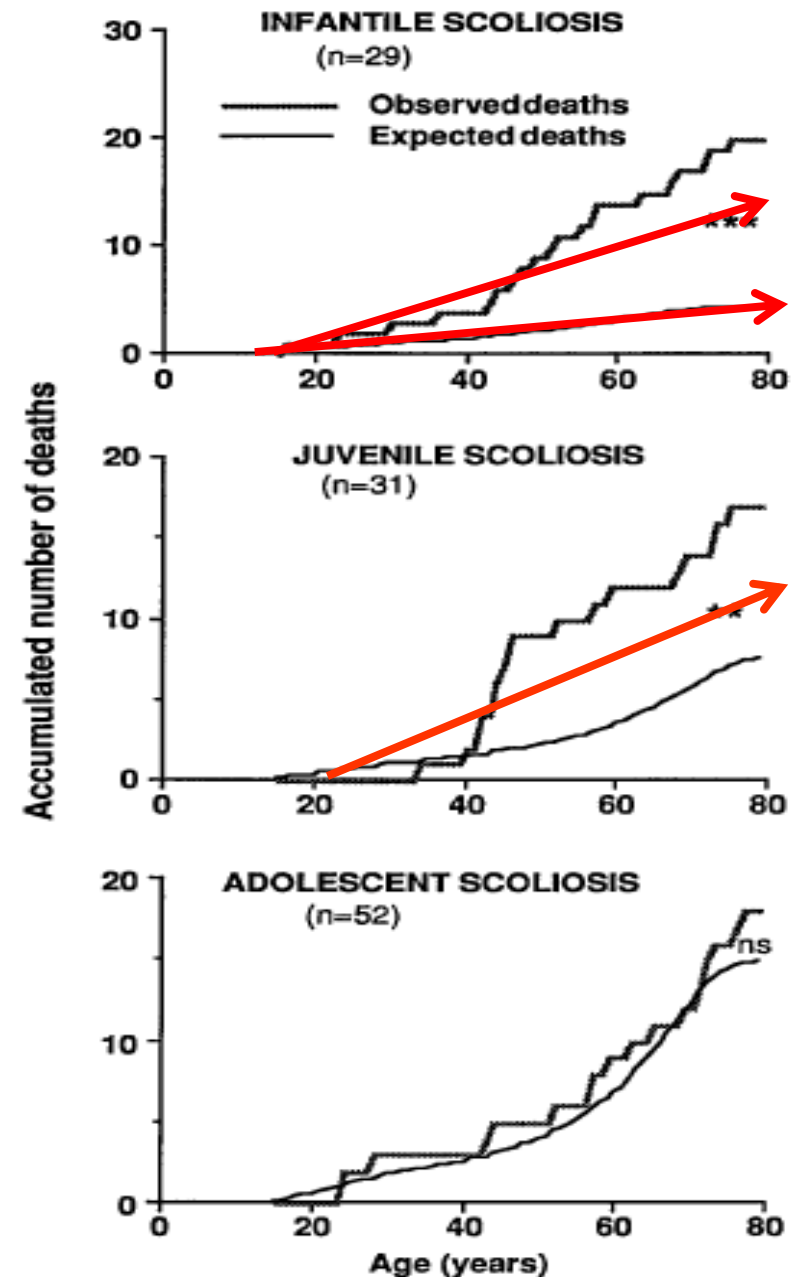


Figure 2. Cumulated number of observed deaths in patients with infantile, juvenile and adolescent scoliosis, and expected deaths.

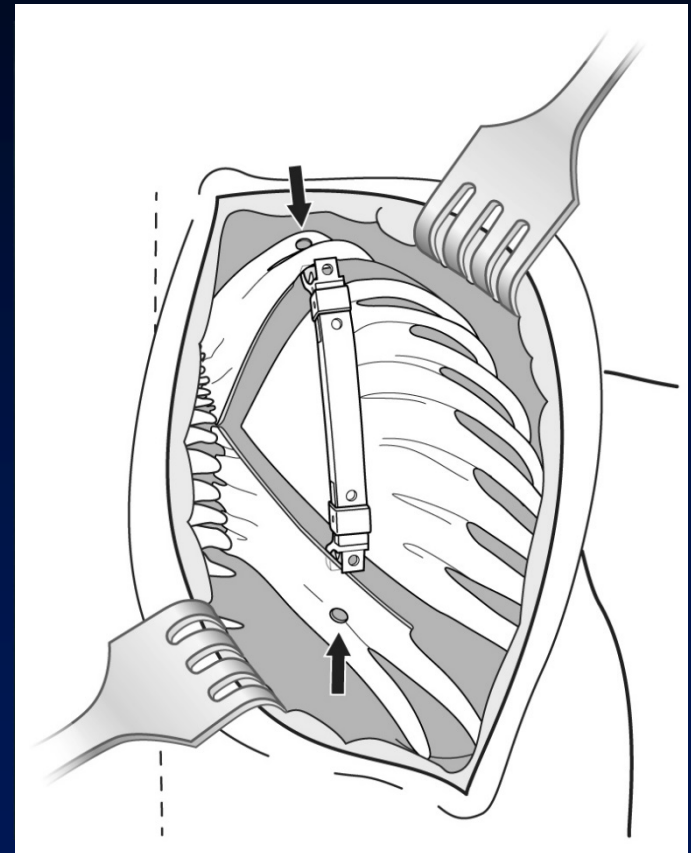
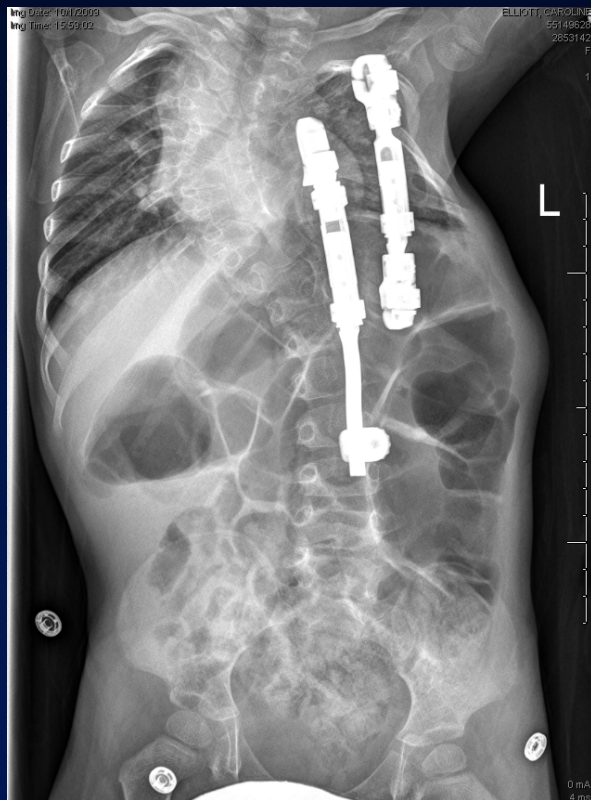
Long term VEPTR treatment of Jarcho-Levin Syndrome

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

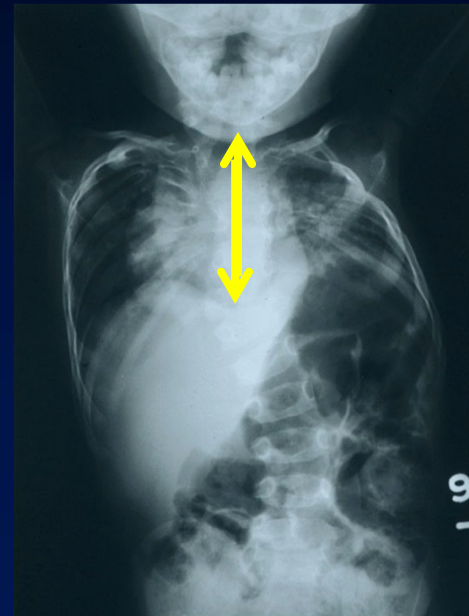
- SCD



STD

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

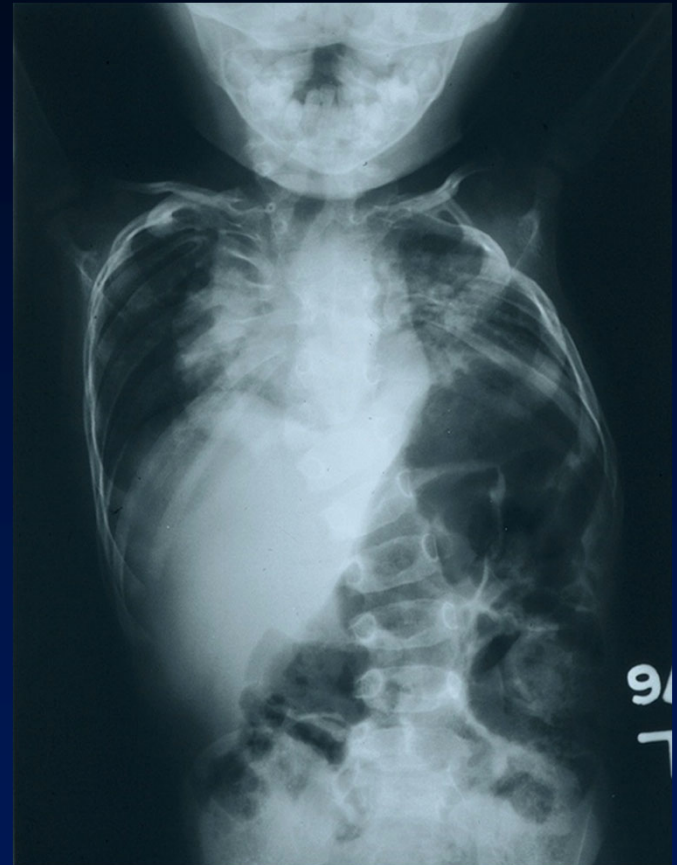
- 3 pts pre-op
- 1 pt at f/u

FVC

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

(Natural Hx, < 30% predicted,

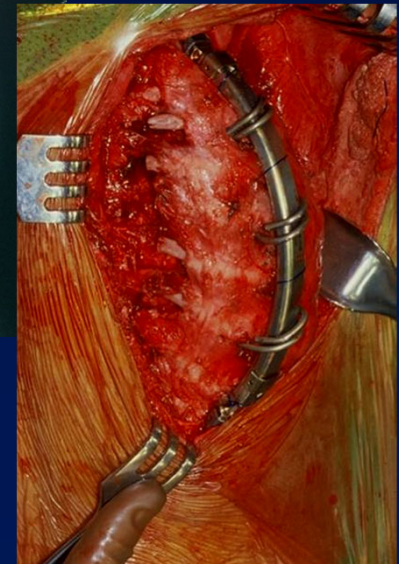
Ramirez, et al., JBJS 2007)



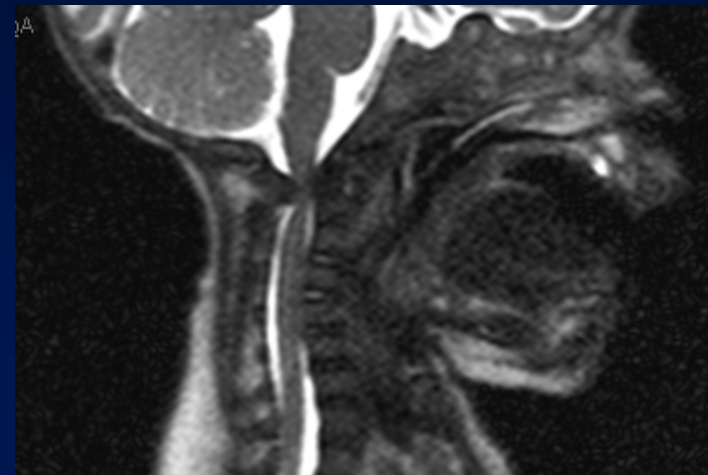
Long Term f/u of VEPTR treatment for Jeune Syndrome

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

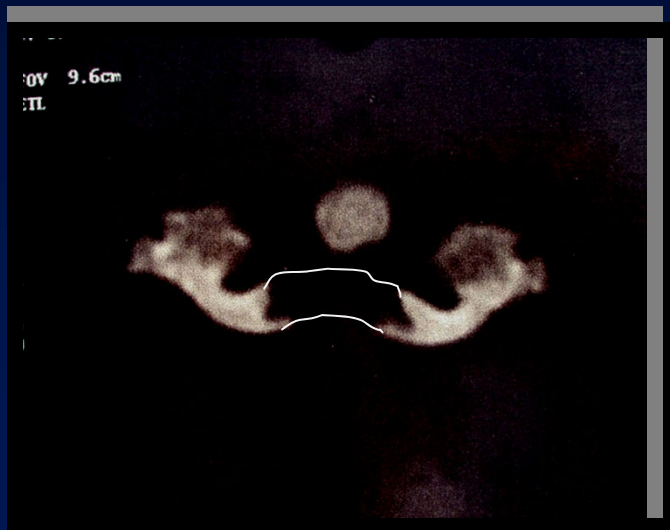


C1 cervical stenosis in 16%

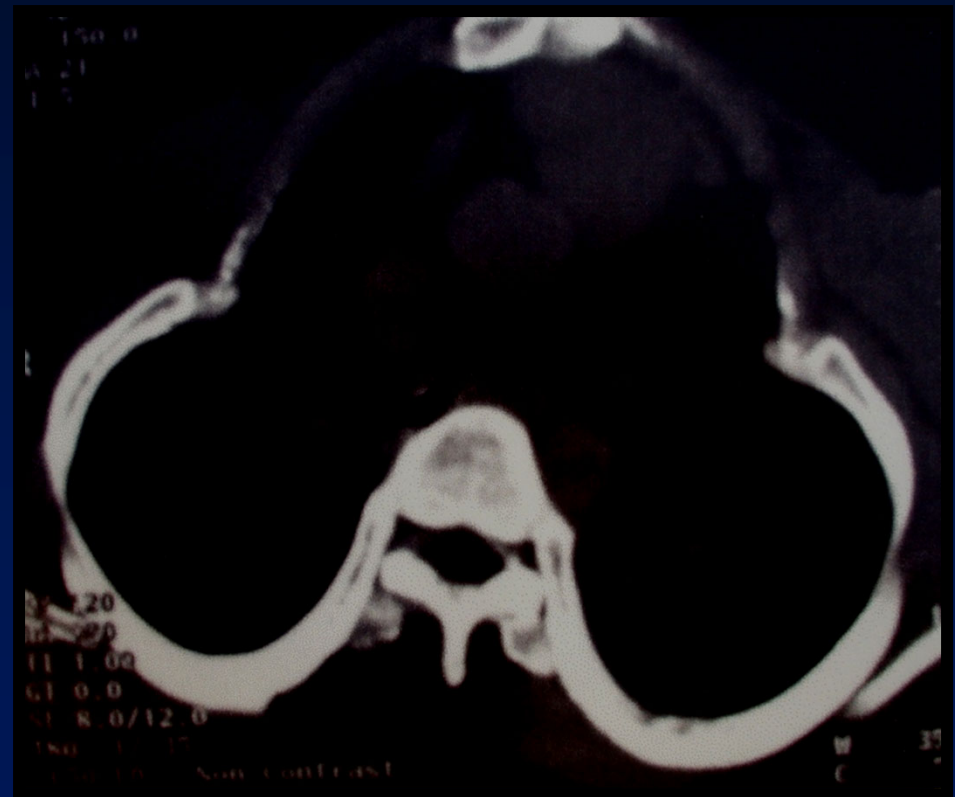


Jeune's Syndrome

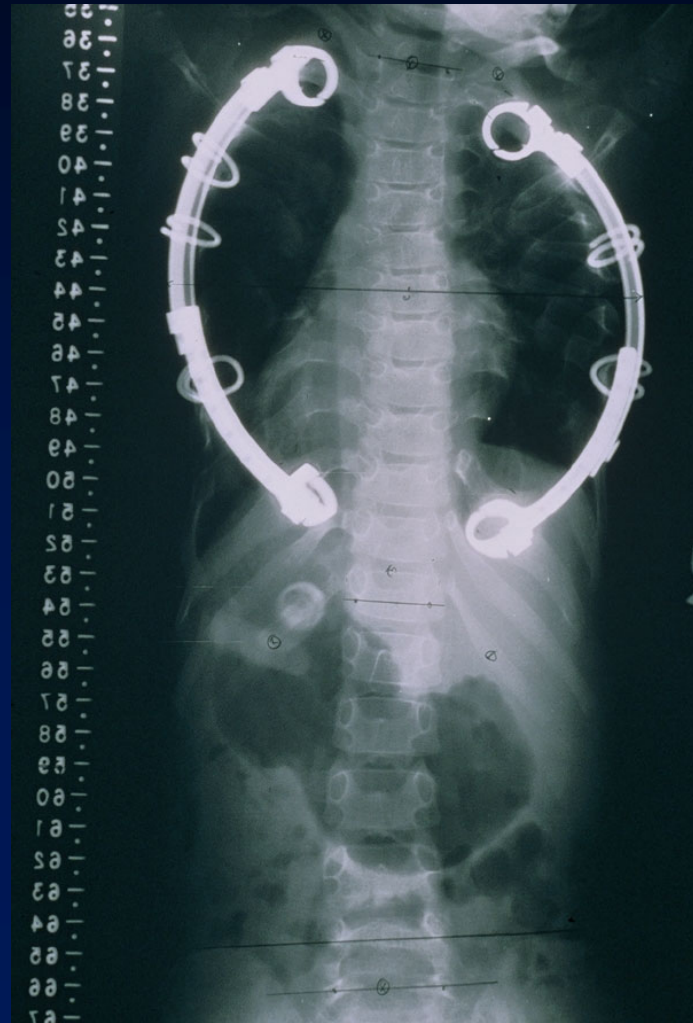
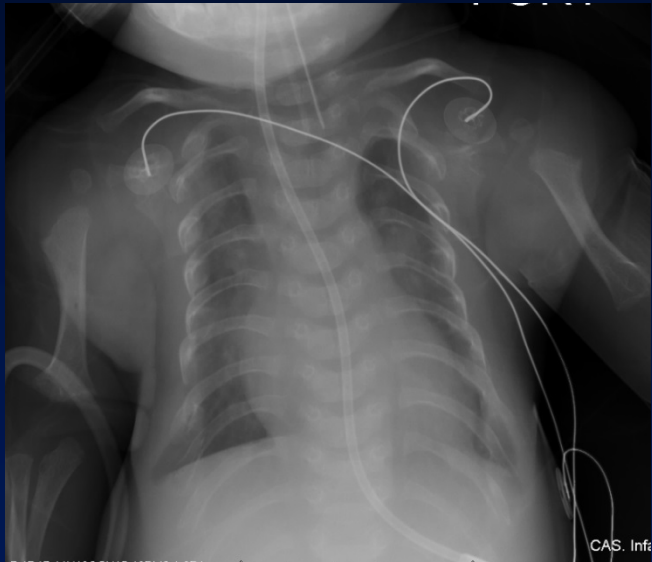
C-1



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

– Oberklaid et al., *Arch Dis Child*. 1977, O'Connor MB, et al. *Postgrad Med J*. 2008

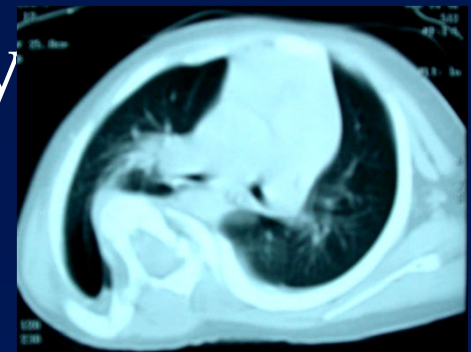
- VEPTR treated (n= 22)

- 4 pts had early demise

- 2 with multi-system disease

—68 % are survivors

f/u 8.4 years (2.3–15.6 y)

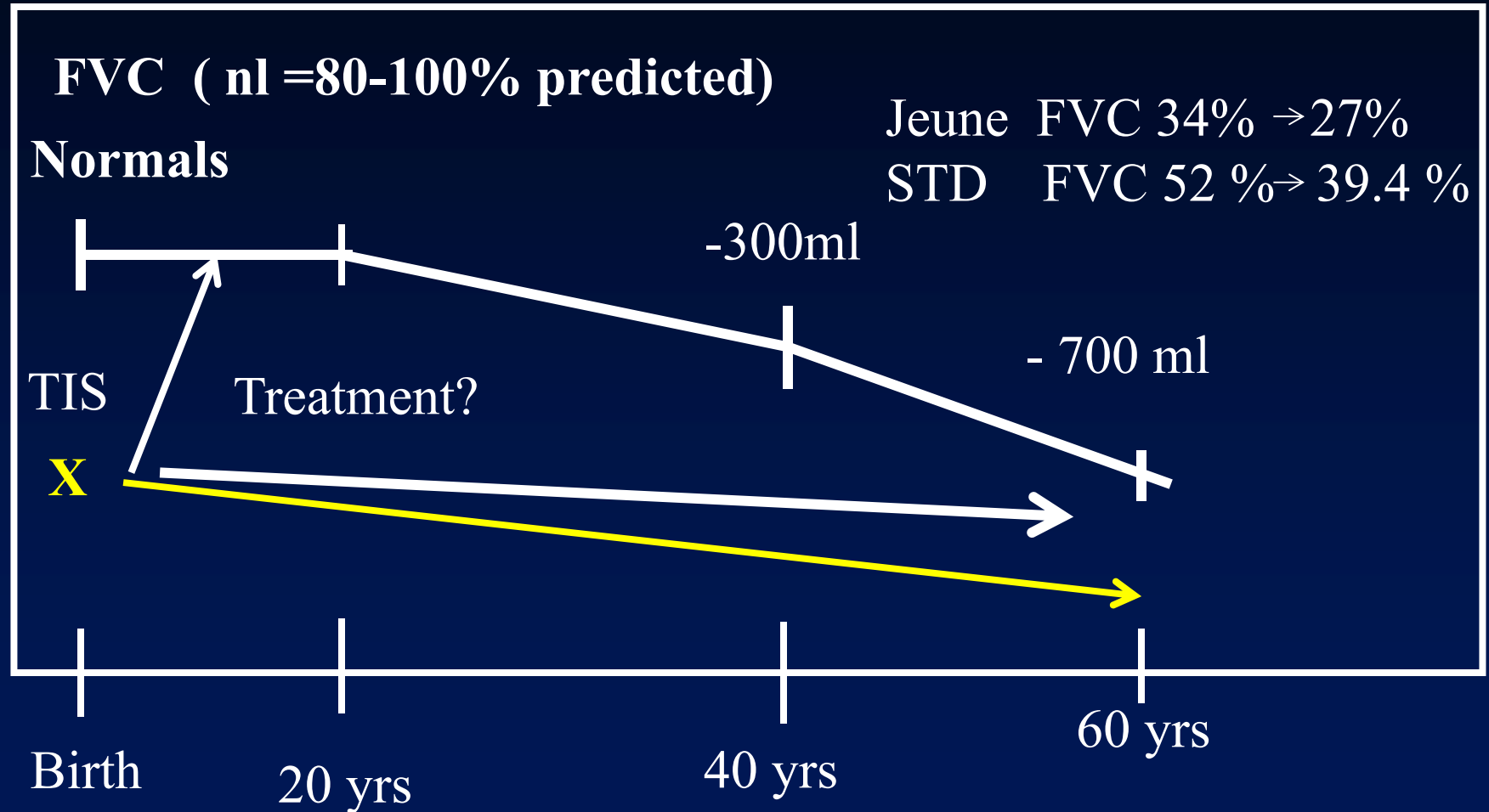


Can we cure Thoracic Insufficiency Syndrome?

no



What is the reality of long term FVC?



SMA II/III: natural history and fusion FVC

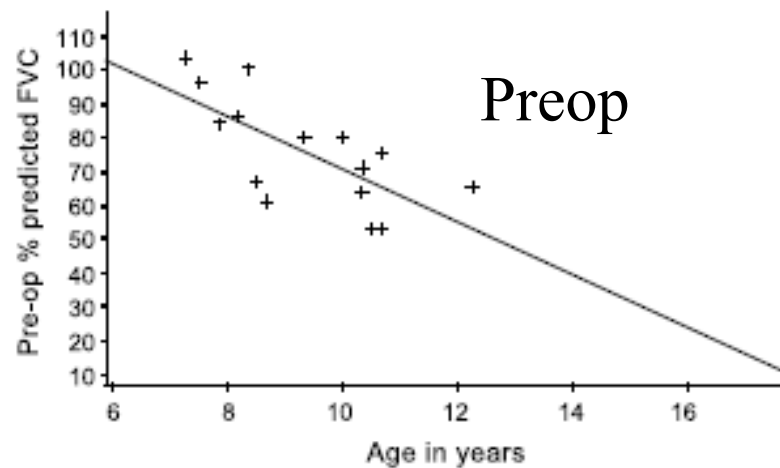


Fig. 1 Pre-operative percentage of predicted forced vital capacity (FVC) versus age in years.

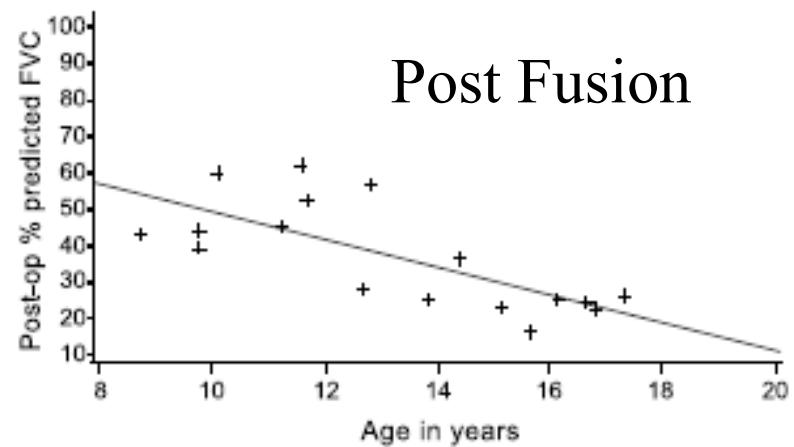
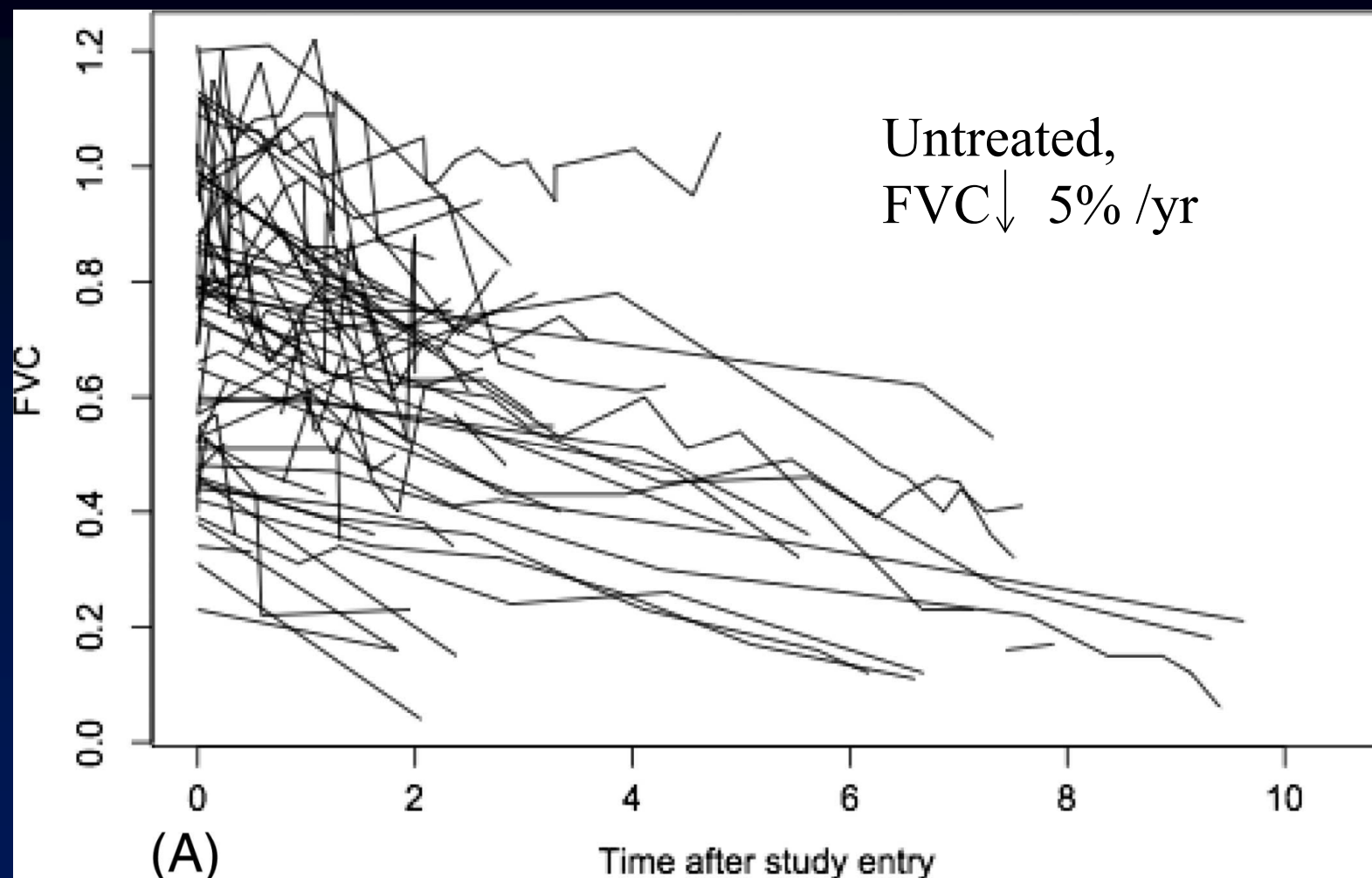


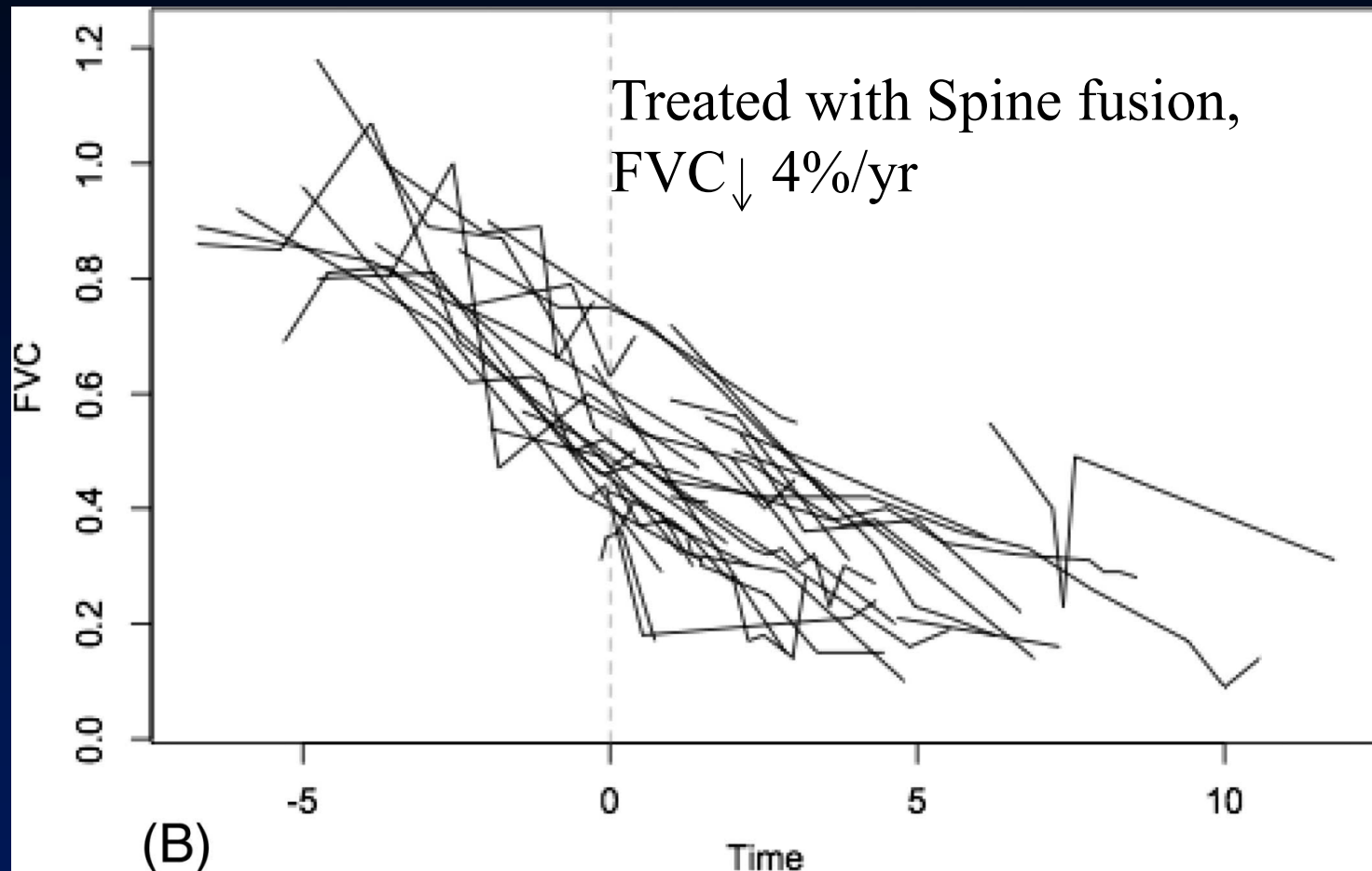
Fig. 2 Post-operative percentage of predicted forced vital capacity (FVC) versus age in years.

-Chng, et al, J Ped Child Health, 2003

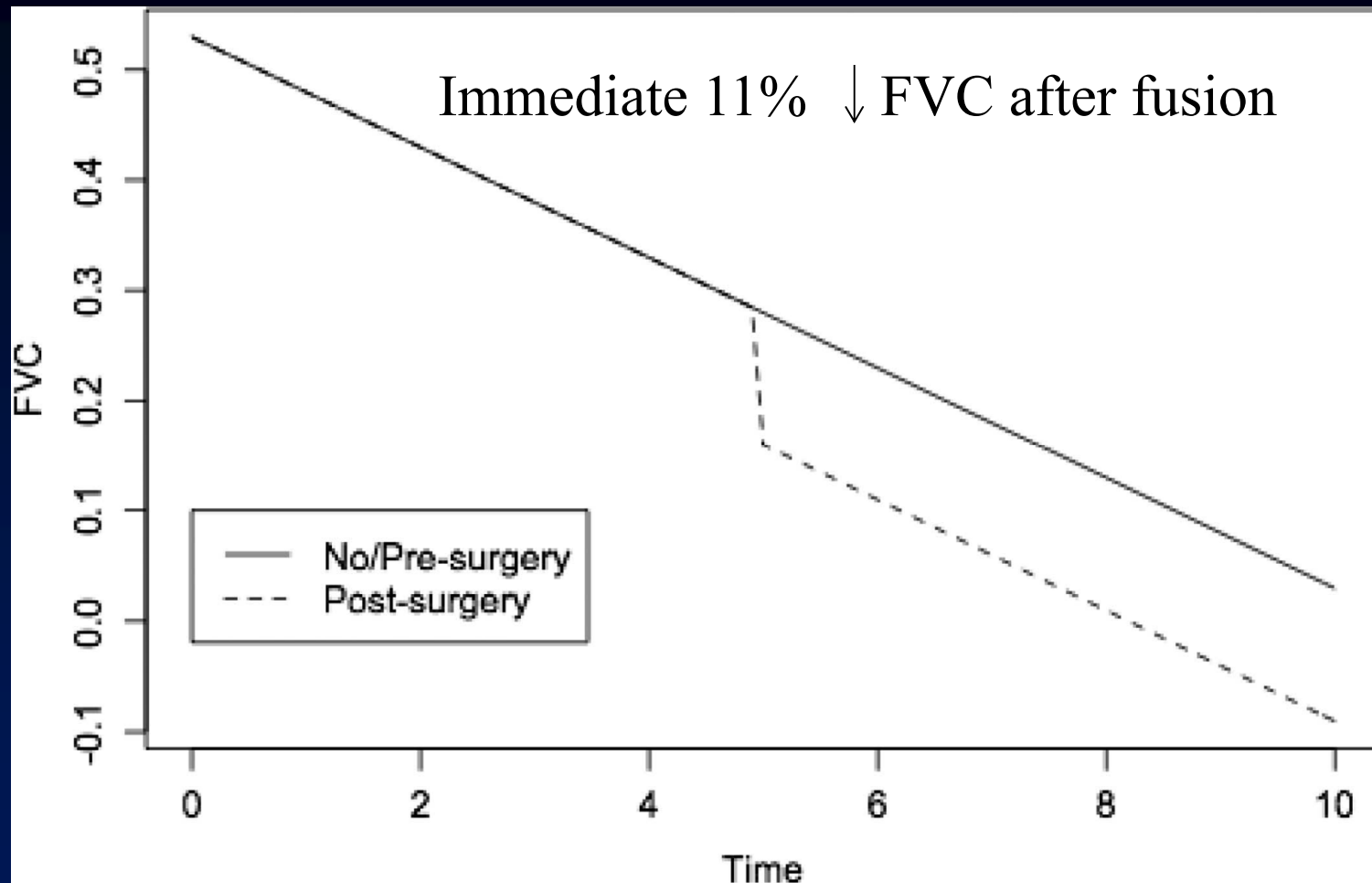
Duchenne MD and Scoliosis



Duchenne MD and Scoliosis



Duchenne MD and Scoliosis



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



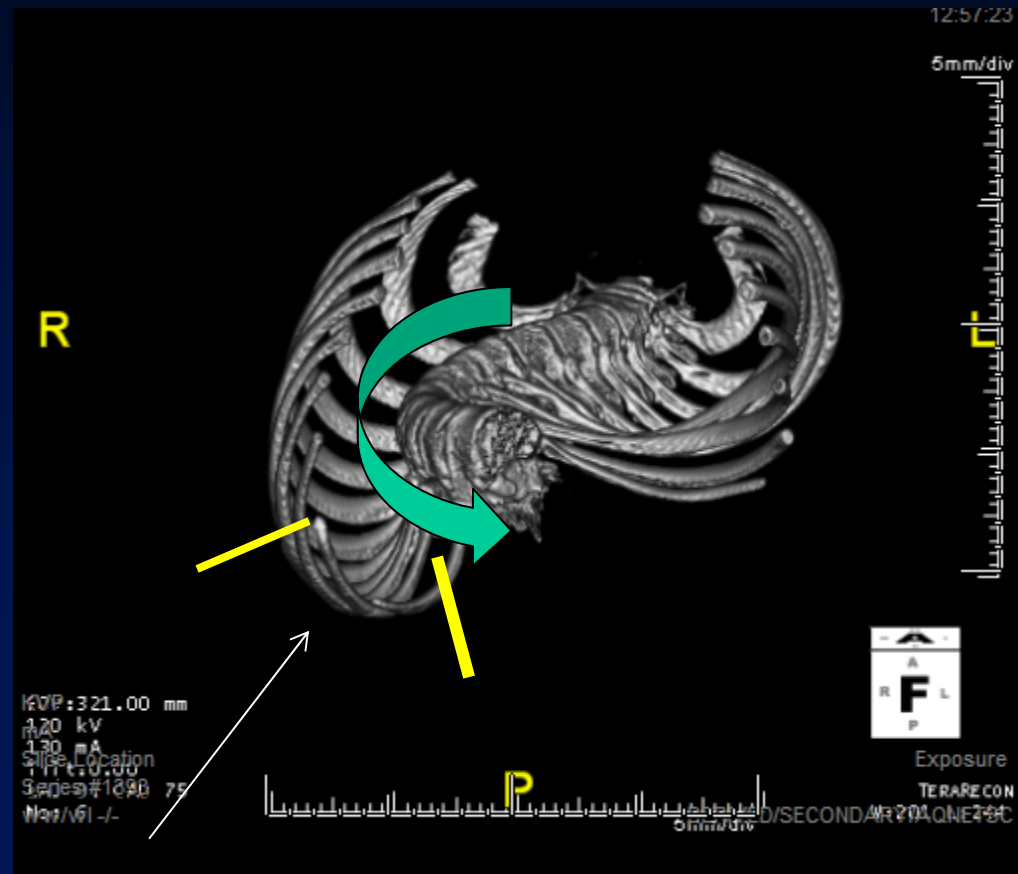
Having instrumentation:
Great feeling!

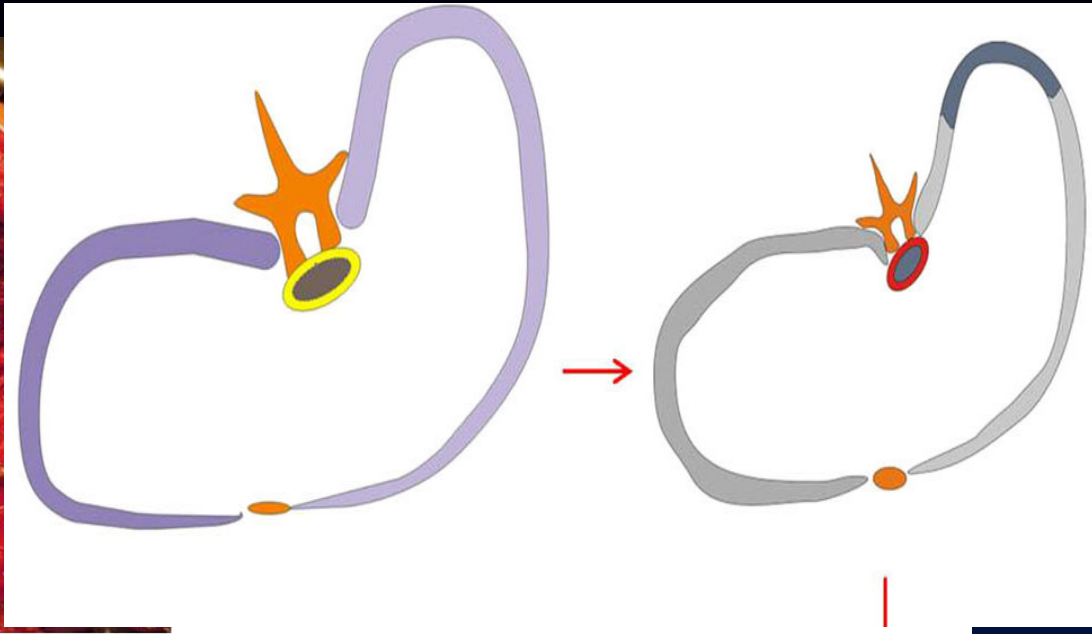
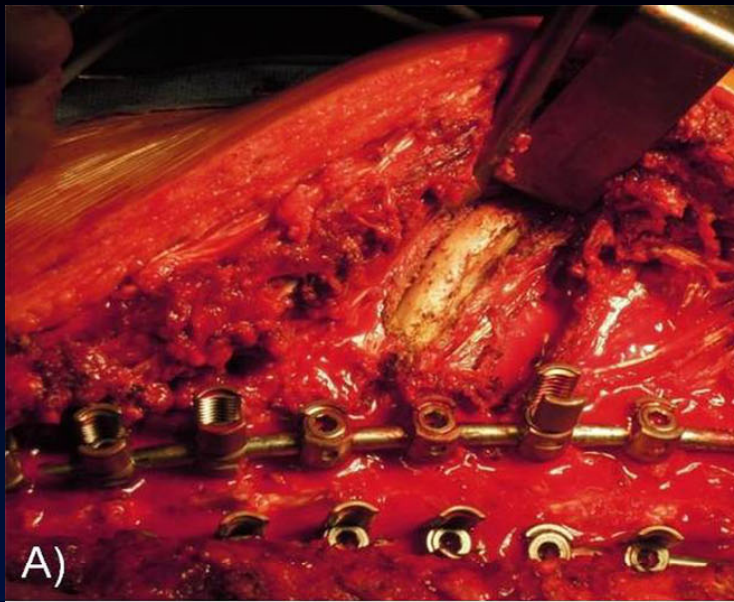
Really understanding the disease
you are trying to treat:

Priceless...

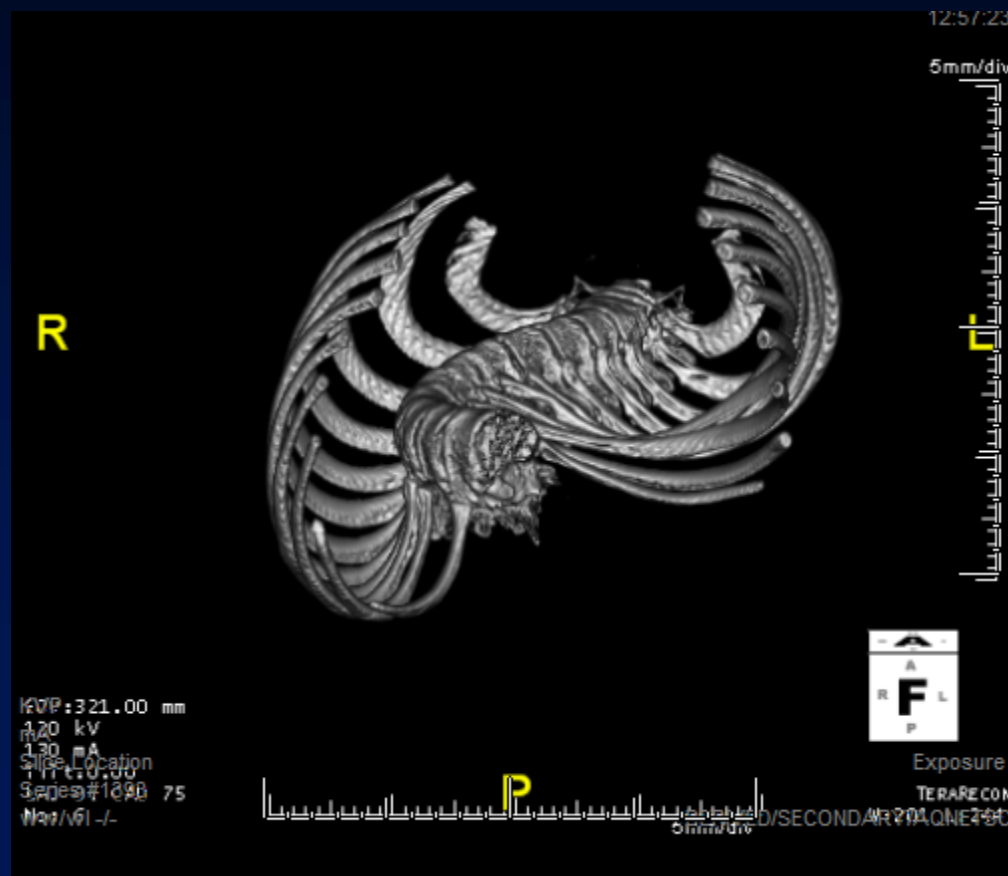


The Rib Hump: Thoracoplasty





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)

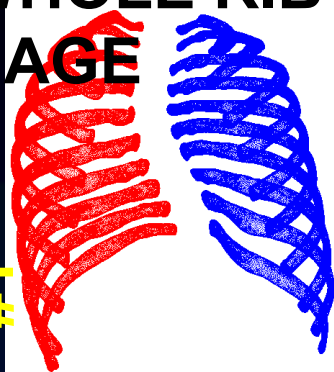
Mean age: 14.15 ± 1.41 years

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

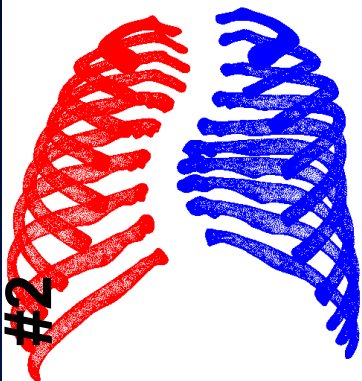
SUBJECT

#1

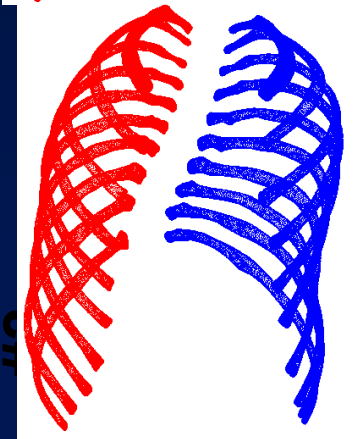


SUBJECT

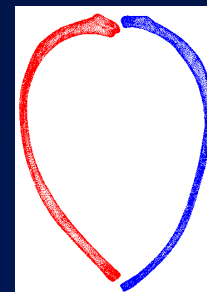
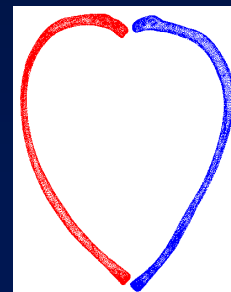
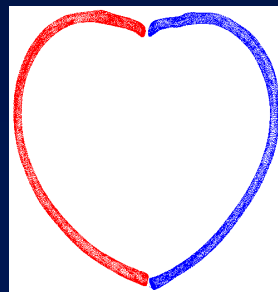
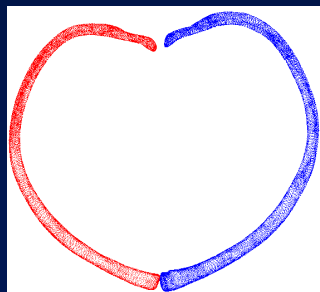
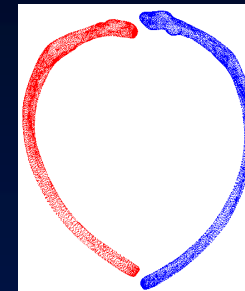
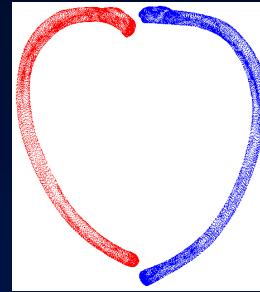
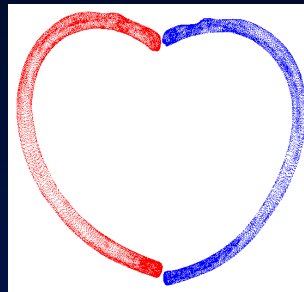
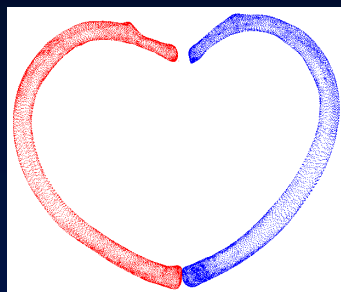
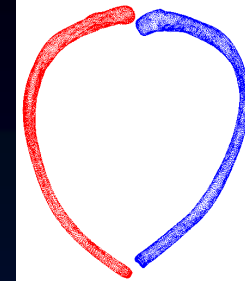
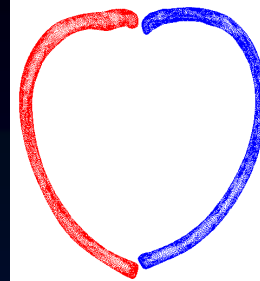
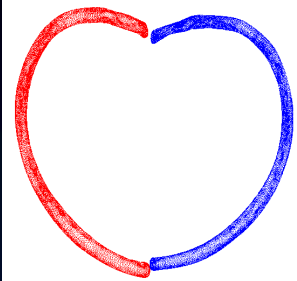
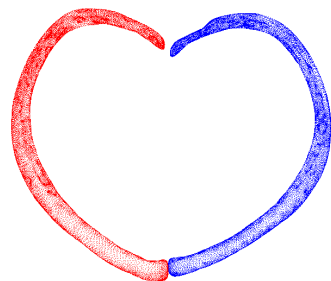
#2



SUBJECT



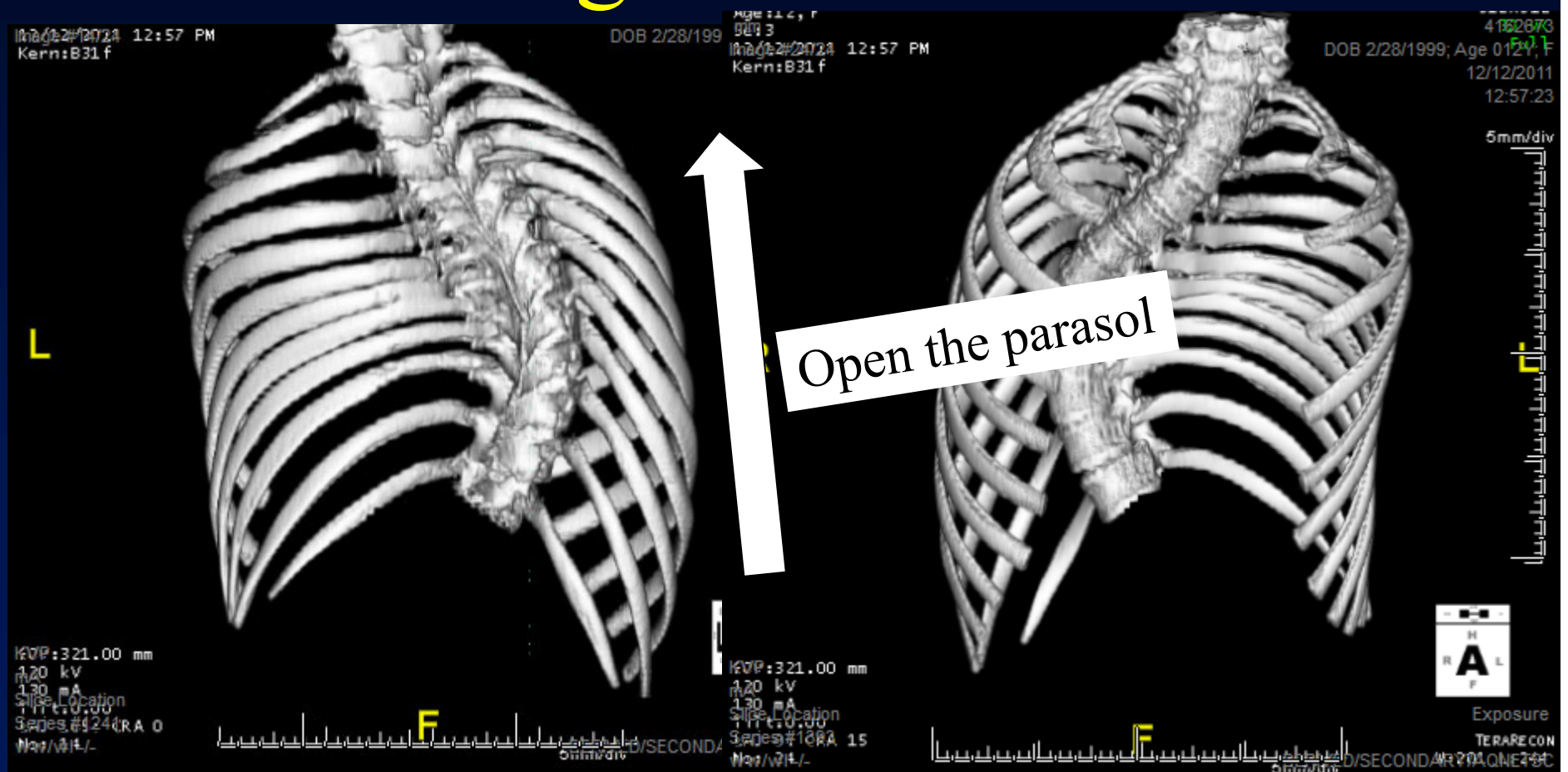
CTIS 



RED – RIGHT
RIB

BLUE – LEFT
RIB

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

Reconstructive

Salvage



Surgical Site Infection Rates

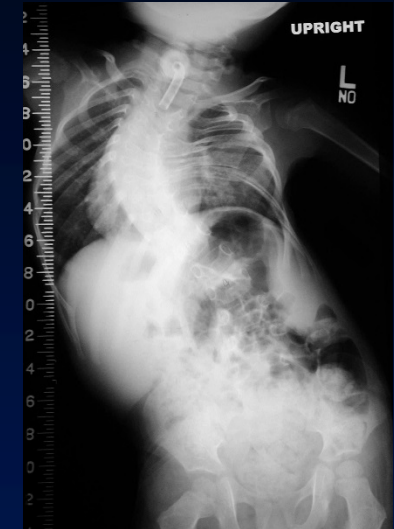
Mackenzie, et al, JBJS, 2013 1347 procedures

Growing Instrumentation constructs

	insertion	revision/lengthening
– Syndromic	28%	8-10%
– NM	10%	6.8%
– Congenital	7%	2.8%
– Infantile/Juvenile Idiopathic	0%	3.6-29%

(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.
 - Fernell E et al. *Acta Paediatr Scand* 1990;79:1080-6.
 - Kestle J et al. *Pediatr Neurosurg* 2000;33:230-6.

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
 - Pudenz RH: *Surg Neurol* 15:15–26, 1981
- Shunt-related hospital admissions account for \$1.4–2.0 billion in hospital charges yearly
 - Patwardhan Rvet al. *Neurosurgery* 56:, 2005

Despite complications , it's
better than natural history



The Timing of surgical treatment?

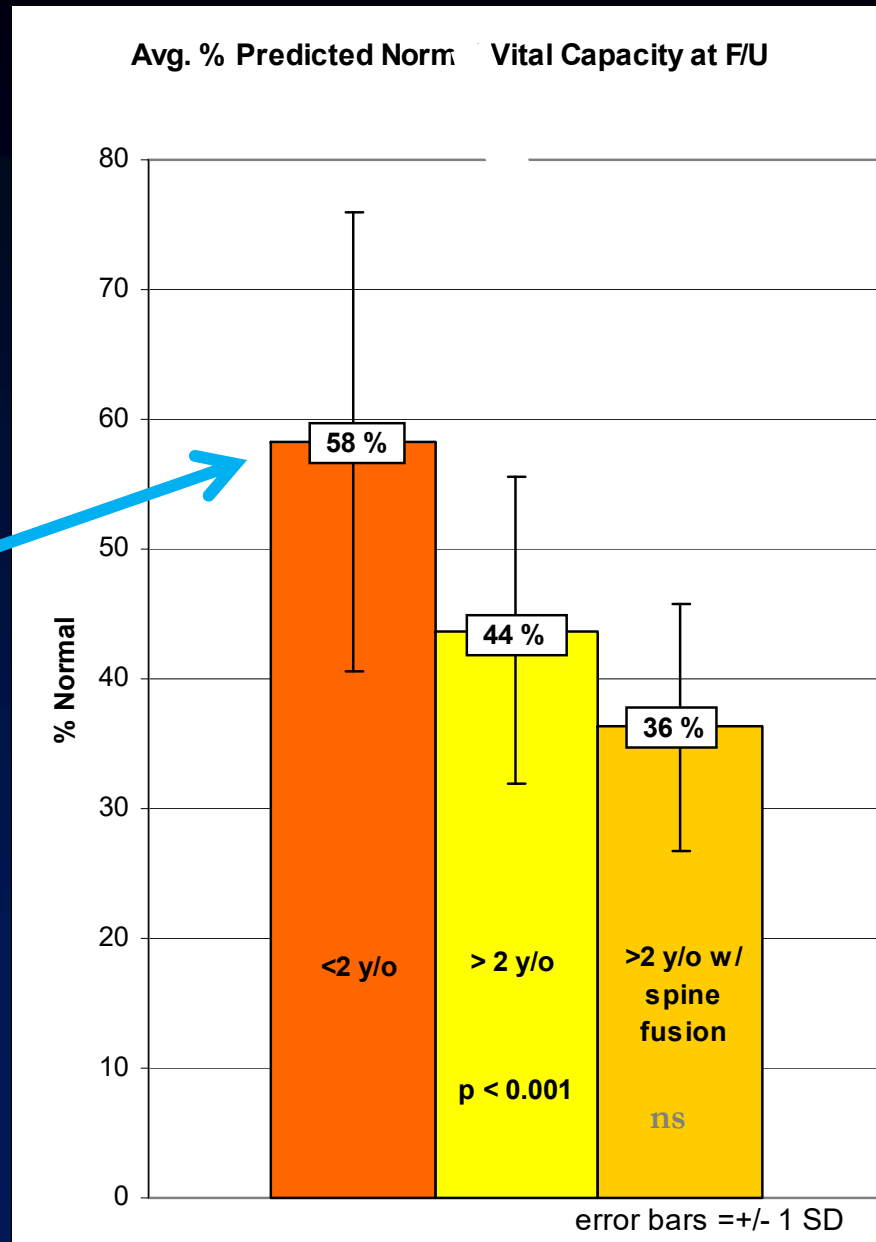
Delay to avoid complications of long term treatment?



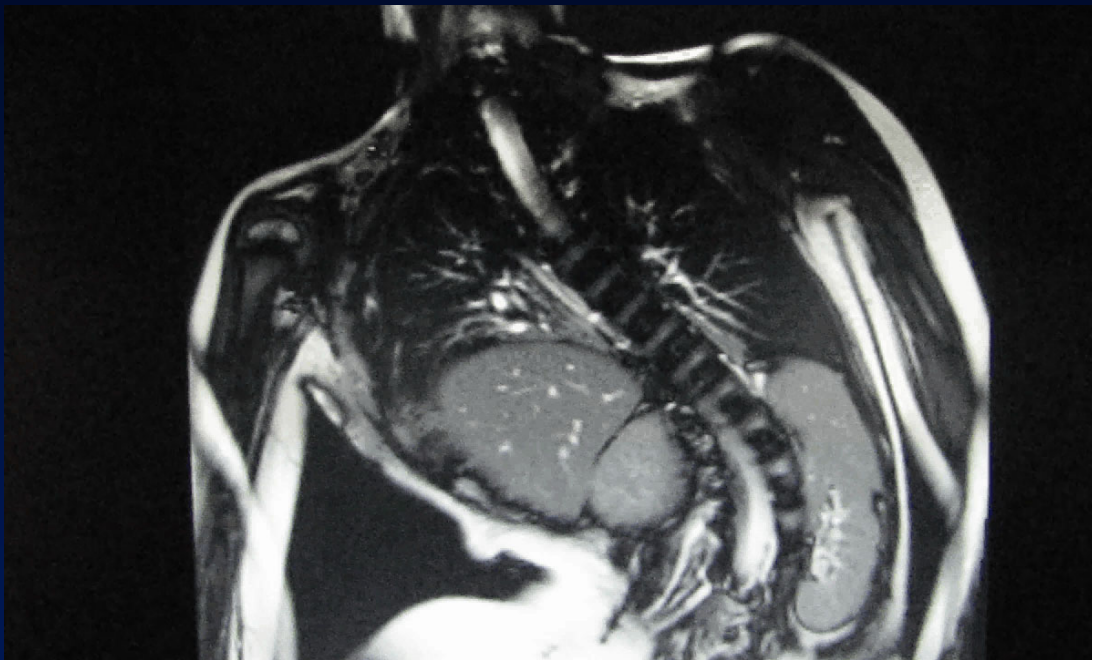
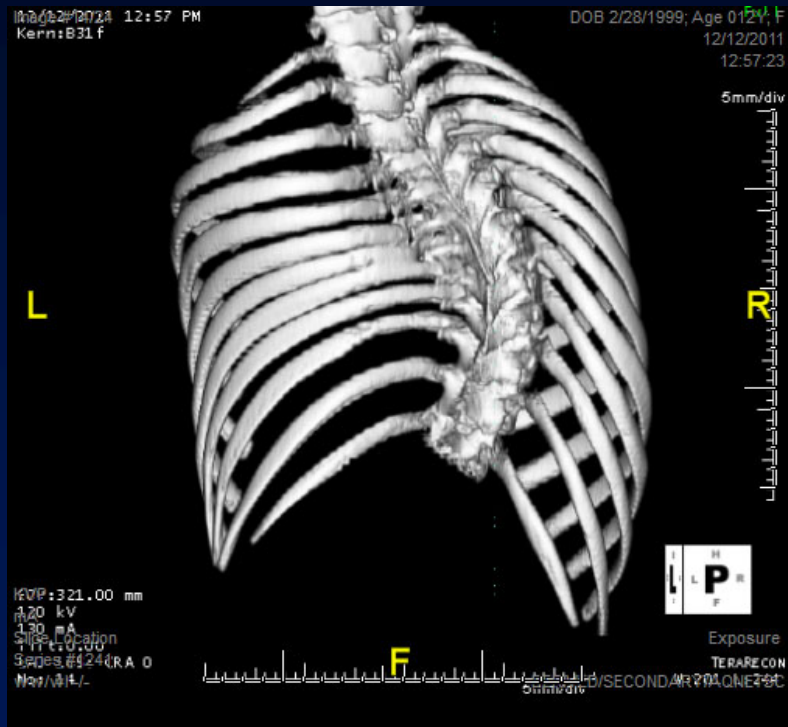
Avg. Predicted Normal
Vital Capacity at follow-
up: VEPTR tx'd Fused
ribs and congenital
scoliosis
< 2 yr at VEPTR
surgery

>2yr at VEPTR
surgery

>2 yr with hx fusion



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!

