

# Outcome Measures in Early Onset Scoliosis

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# Disclosures

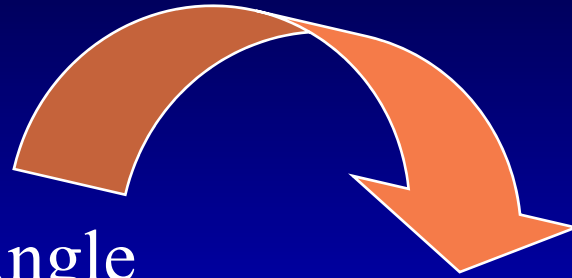
- I am a consultant for Stryker Spine and Biomet Spine
- Royalties from Biomet Spine
- Receive Divisional support from Medtronic, Biomet, AO Spine
- Almost nothing I am discussing is approved for the indications that I am using it



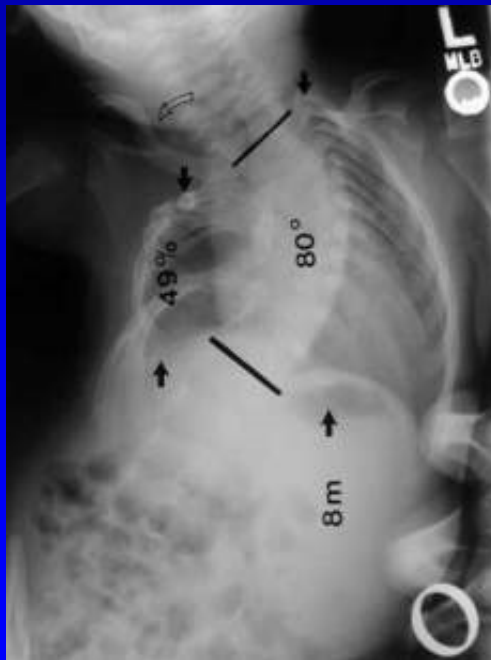
No Conflicts Related to Presentation

# ORTHO-PAEDICS

## Is a “straight child” enough?



- Cobb Angle



- Pulmonary Function
- Quality of Life
- Life Expectancy



# Outcomes: Compared to What ?

- Natural History
- Early Fusion
- Comparison of various techniques eg GR vs VEPTR
  
- How to measure ?
  - Life expectancy
  - Pulmonary Function
  - Quality of Life
  - Burden of Care
  - Psychosocial Effects of Repetitive Surgery

# What We Know

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- **EOS can be a bad disease**
- **Outcomes in EOS are hard to measure**
- **Evidence is difficult to accumulate**

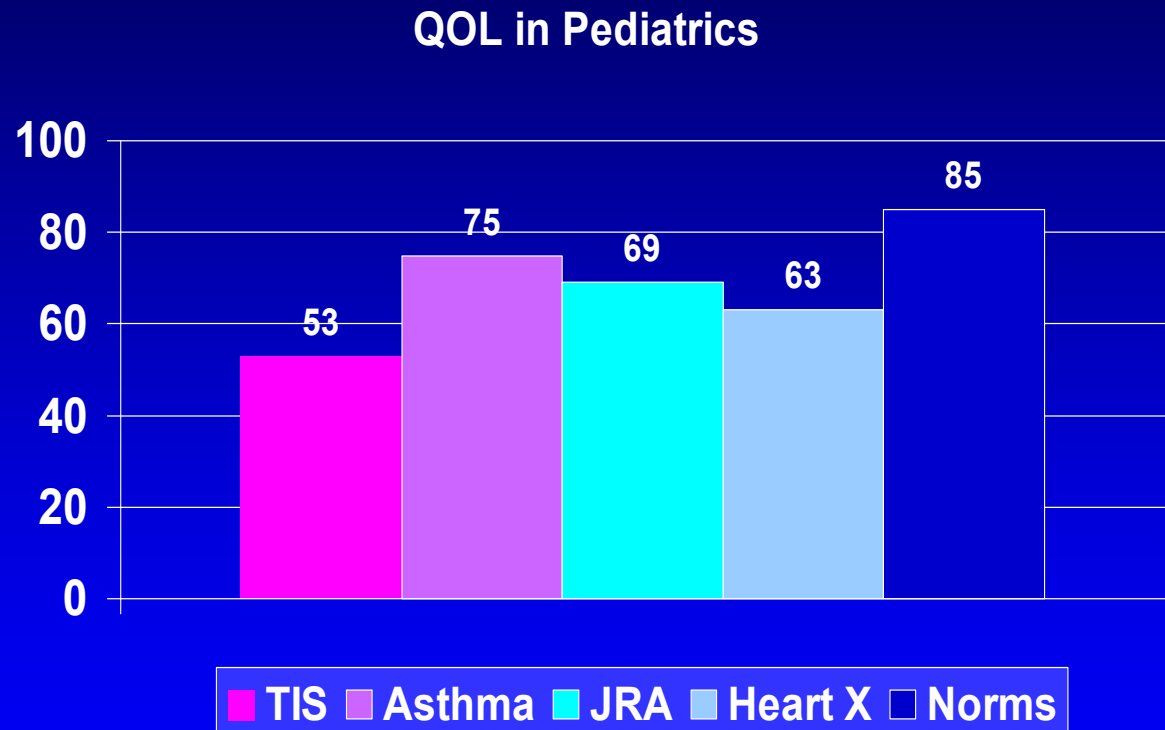
# Early Spine Fusion is Associated with Adverse Pulmonary Outcome

- **Goldberg CJ, et al.** Respiratory function and cosmesis at maturity in infantile-onset scoliosis.
- **Emans JB, et al.** Earlier and More Extensive Thoracic Fusion is Associated with Diminished Pulmonary Function: Outcomes after Spinal Fusion of 4 or more Thoracic Spinal Segments Before Age 5.
- **Karol, L. et al.** The effect of early thoracic fusion on pulmonary function in non-neuromuscular scoliosis.
- **Vitale et al;** Retrospective Cohort Study of Pulmonary Function, Radiographic Measures and Quality of Life in Children with Congenital Scoliosis: An Evaluation of Patient Outcomes after Early Spinal Fusion

# Quality of Life in Thoracic Insufficiency Syndrome

- These scores are among the **lowest observed in pediatrics**

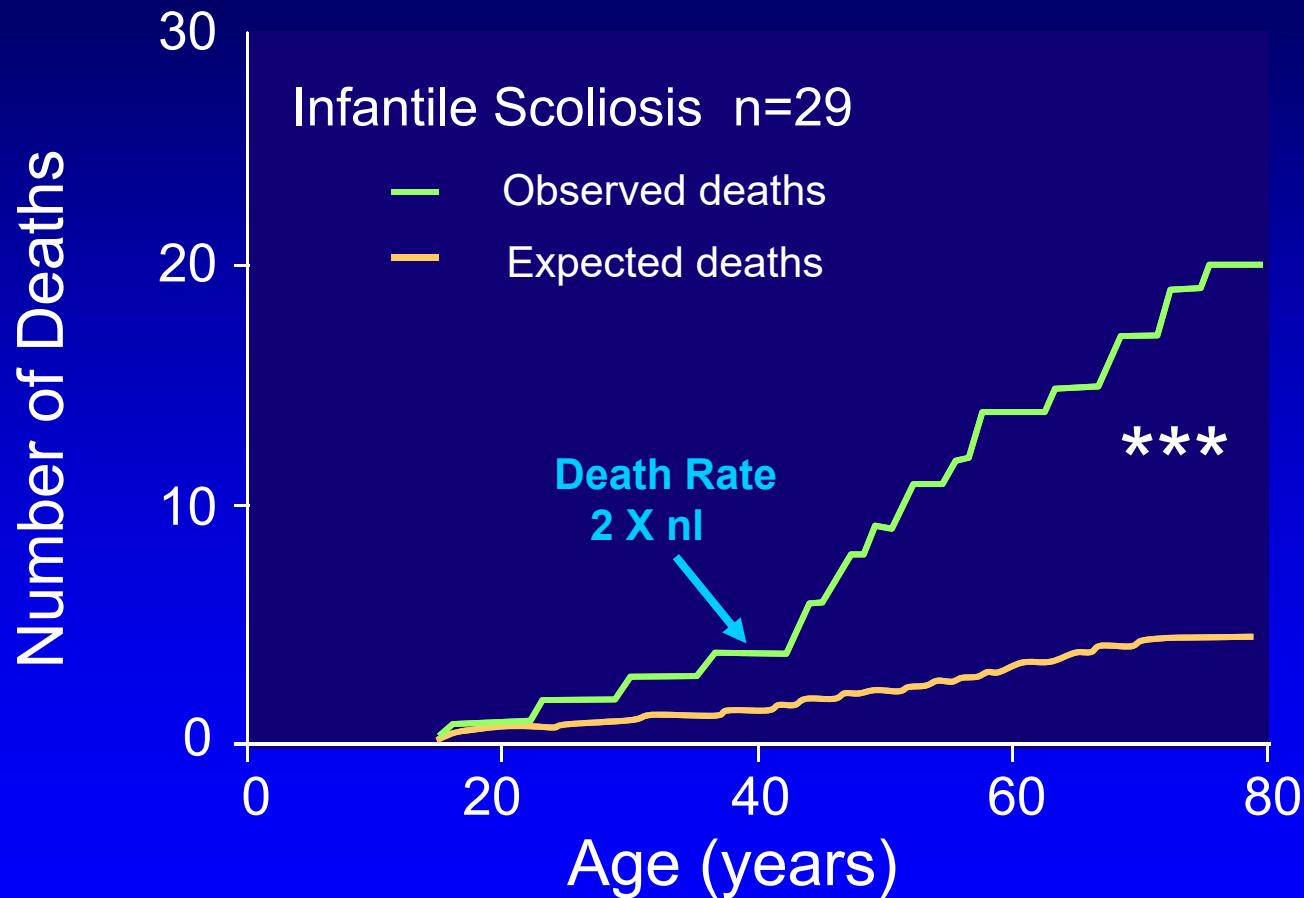
- Asthma
- JRA
- Heart transplant



What We Know

# Natural History is Bad

## Infantile Idiopathic Scoliosis



Pehrsson, Larsson, Oden & Nachemson, Spine, 1992

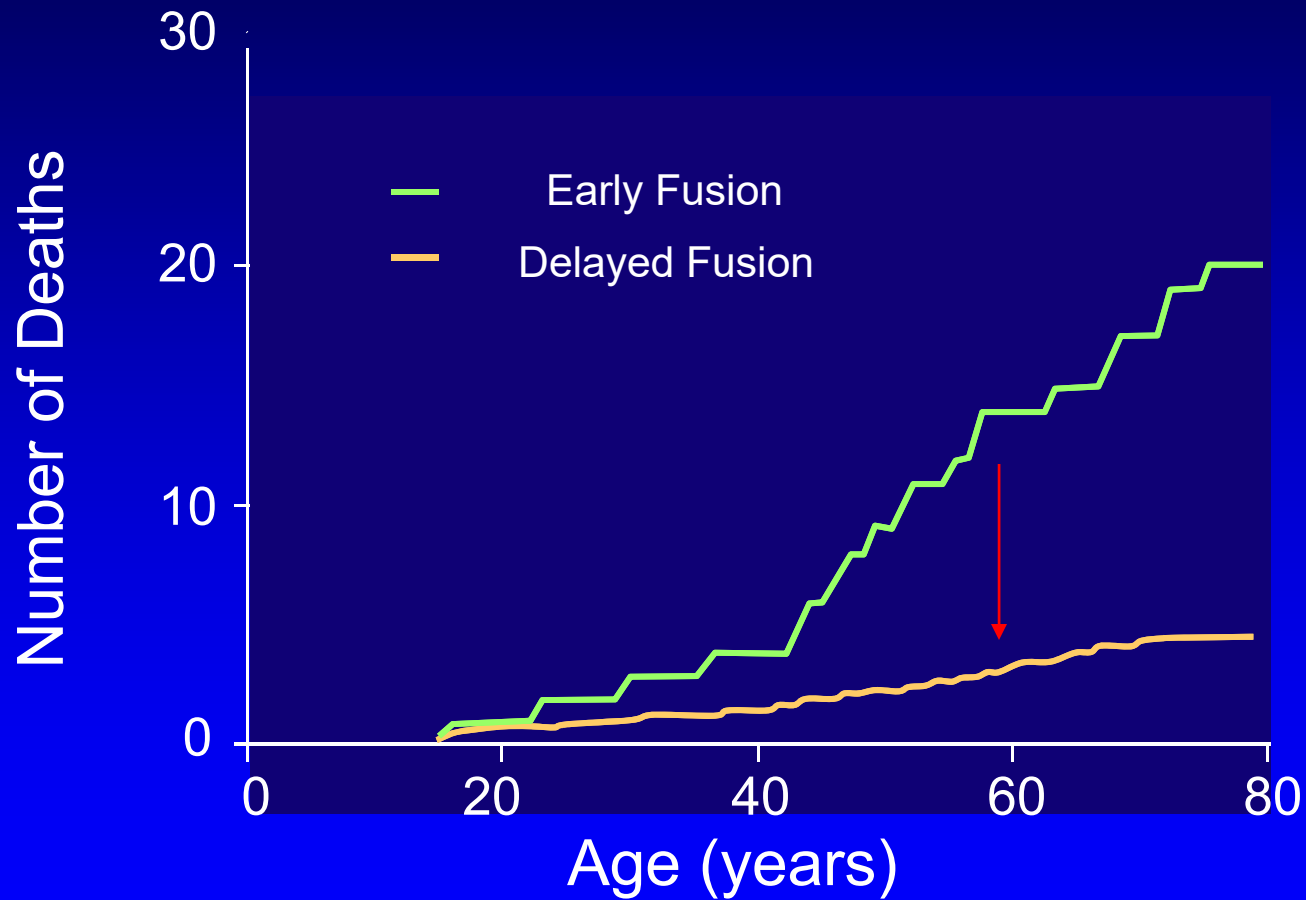
What We Know



# RCT: Natural History of EOS VS NonFusion

Natural Hx n=416

Growth Strategies/ Delayed Fusion N = 405



Vita ~~et al, NEJ~~, 2008

What We Don't Know

# The Challenge to Assessing Outcomes: The Way Forward

EOS is heterogenous disease

- Need EOS Classification system



Rare Disease

- Need to improve infrastructure for multicenter research (challenge to study groups)

Standardized Measures of Outcome Not Available

- Need to Develop SRS-like measure for EOS
- QOL
- Disease Specific Traditional Measures

# Ideal Outcome Characteristics

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- Consensus (Buy in)
- Reliable
  - Inter and Intraobserver Reliability ( $\kappa$ , ICC)
- Valid
  - Measures what its supposed to
- Responsive
  - Reflect changes with treatment

# Ideal Outcome Characteristics

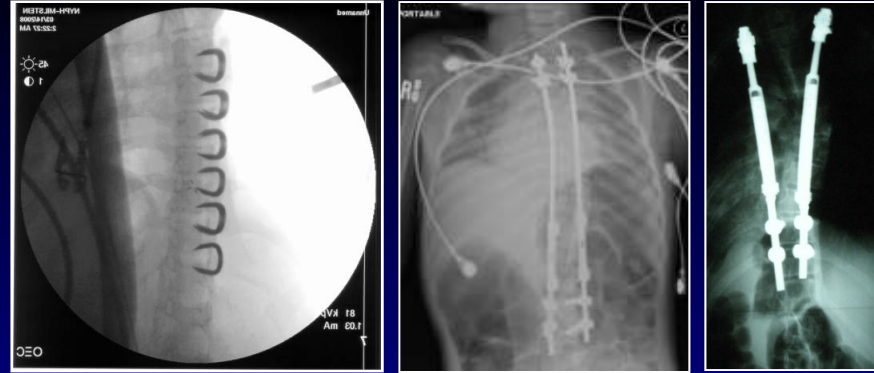
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## Modular

- QOL
- Pulmonary
- Radiographic
- Caregiver burden
- Complications

## Feasible

- Burden on staff and clinician, Risks to patients (e.g. radiation)



## Health Related Quality of Life in Children with Early Onset Scoliosis: *Validation of a Disease Specific Instrument*

# ICEOS 2008



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# EOS DSI: 12 Domains and 24 Questions

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## EOS DSI

- General Health
- Pain
- Pulmonary Function
- Physical Function
- Daily Living
- Fatigue
- Emotion
- Parental Burden
- Financial Burden
- Surgery
- Satisfaction
- Treatment Outcome

OFFICE USE ONLY		Study ID:	Date: / /	
<b>General Health</b>				
1. In general, you would say your child's health has been:				
Poor	Fair	Good	Very good	Excellent
2. How often has your child missed school due to his/her health condition?				
All of the time	Most of the time	Some of the time	A small amount of time	None of the time
<b>Pain/Discomfort</b>				
3. How often has your child had pain/discomfort?				
All of the time	Most of the time	Some of the time	A small amount of time	None of the time
4. How often did pain/discomfort interfere with your child's physical activities?				
All of the time	Most of the time	Some of the time	A small amount of time	None of the time
5. How often did pain/discomfort interfere with your child's sleep?				
All of the time	Most of the time	Some of the time	A small amount of time	None of the time
<b>Pulmonary function</b>				

# EOS DSI: Next Steps

- Prospective Validation
- Testing of Responsiveness
- Develop Age-based Norms
- Happy to share: email [mgv1@columbia.edu](mailto:mgv1@columbia.edu)

# Psychosocial Effects of Repetitive Surgery on Children with EOS being treated with Growing Instrumentation

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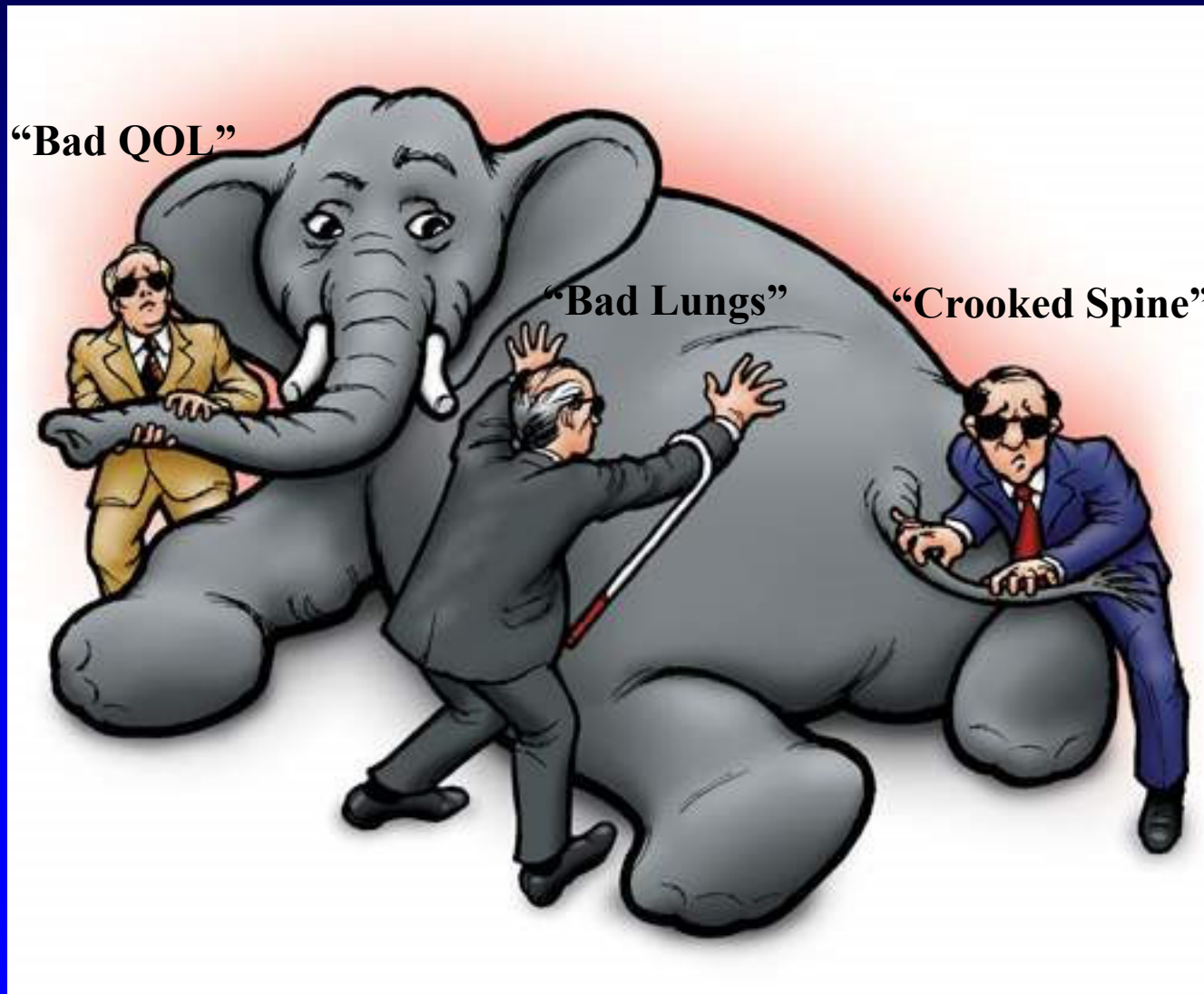
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# Don't be the Blind Man



# Closing Thoughts on Outcomes

- “investigate...and suspend judgment... because we do not have the final truth.”

**Pyrrho (4th-3rd c. B.C.) and the Sceptikoi**

- “primum non nocere” vs Do Something (new)

**Hippocrates**

- “...they also serve who stand and wait.”

**John Milton – *On My Blindness***

- “absence of proof is not proof of absence”

**W Cowper 1875**



# Thank You



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