Which is the Right Assessment Instrument? (What Have We Learned from EOSQ-24)

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-Disclosures-

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CHILDREN'S SPINE STUDY GROUP

EOS Research Platform

Development of a Research Infrastructure Via Six Parallel Efforts

Endpoints	Development/Validation of a Disease-Specific QoL Measure
Equipoise	Identifying Clinical Equipoise in the Field of EOS
Classification-EOS	Development / Validation of Classification for EOS
Standardizing Complications	Standardize Way We Define and Report Complications
Predicting Complications	EOS Risk Severity Score
Clinical Trials	Proximal Anchors: Rib vs Spine – Retrospective and Prospective

Types of Health Outcomes Measures

Clinical

- Labs
- Clinical Events
- Physician Assessments

Economic

- Direct and Indirect costs
- Cost-utility
- Cost-effectiveness
- Cost-benefit

Patient-Based

- Symptom reports
- Health status
- *Quality of Life*
- Patient Satisfaction

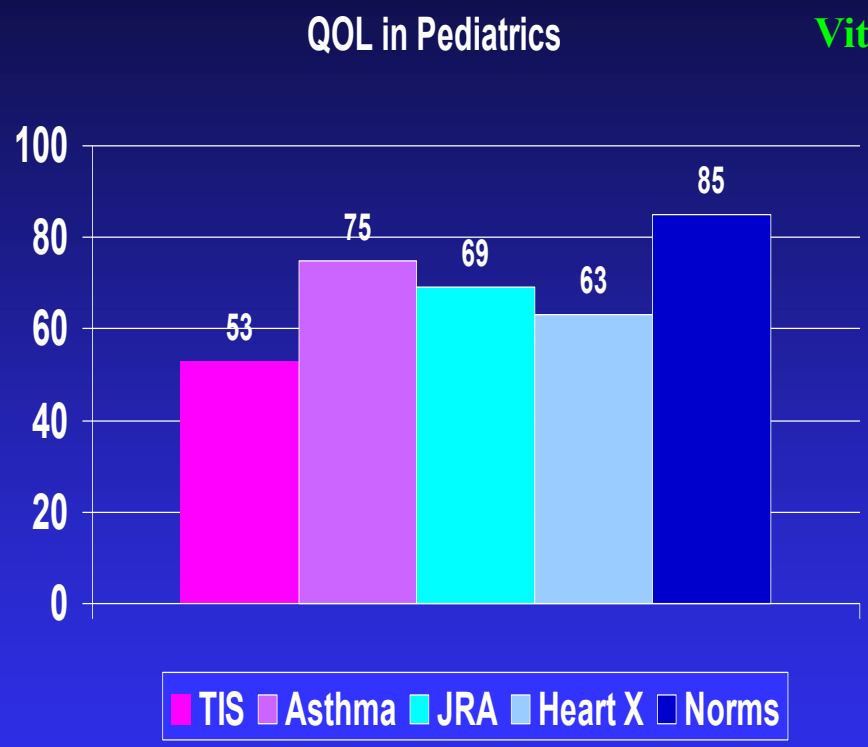
Generic vs. Disease-specific?

- Generic health instruments address larger health constructs and hence their causal links to specific treatment events may be more difficult to detect
  - Eg. CHQ, SF-36
- Disease-specific instrument will vary with the condition being treated, and hence are typically more sensitive to treatment effects
  - Clubfoot DSI (Roye, Vitale, et al, 2001)
  - ACEND (Matsumoto, Vitale, et al, 2011)
  - EOSQ (Corona, Vitale, et al, 2011)

Patient-Based Outcomes in EOS

- Difficult to measure
  - Heterogenous population
  - Significant comorbidities
  - Age is variable
  - Natural History can be subclinical in childhood

Multicenter VEPTR Study--CHQ



Vitale et al. J Pediatr Orthop 2008; 28:239-243

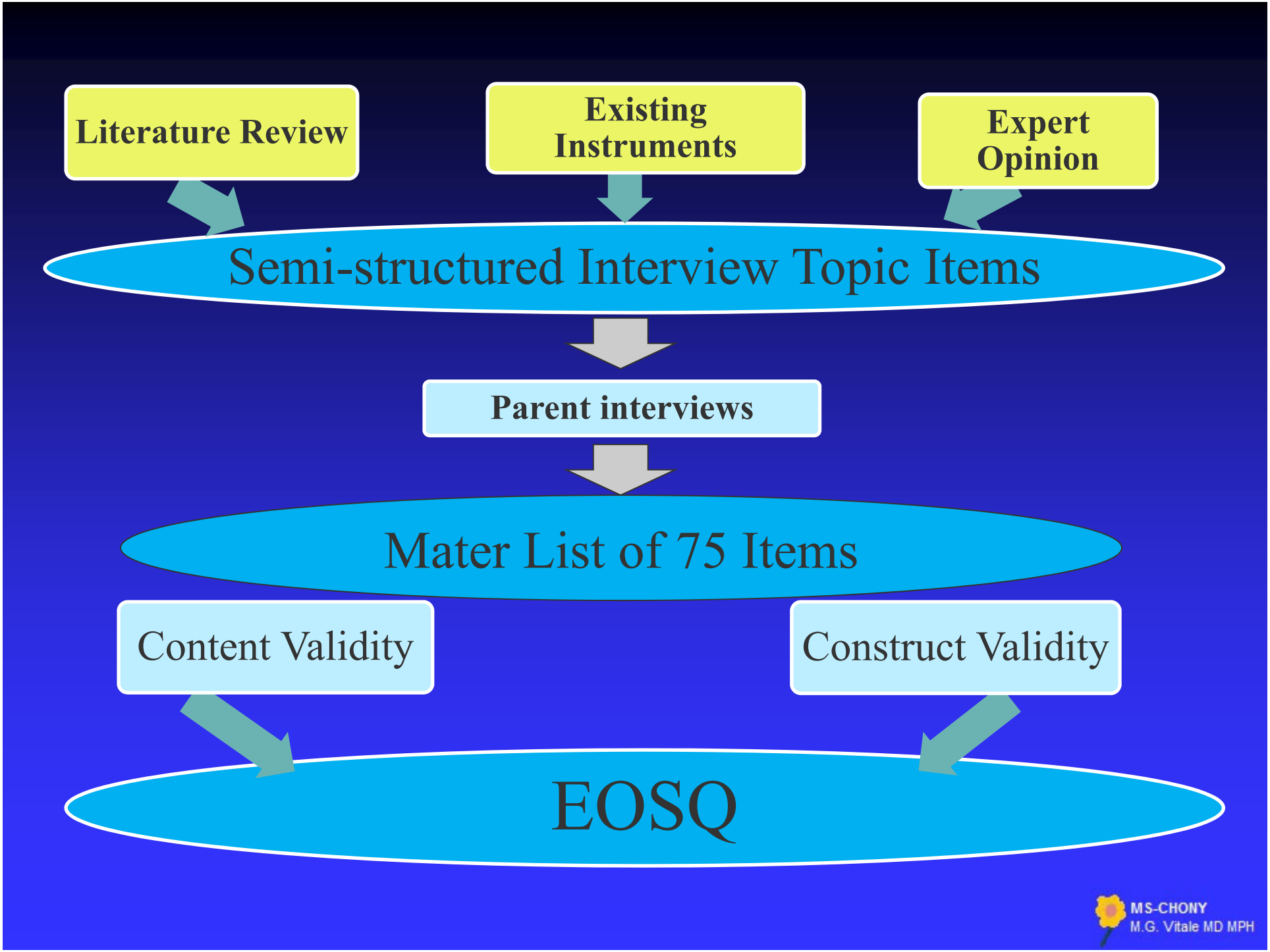
- CHQ scores are lower than those with:
  - Asthma
  - Juvenile Rheumatoid Arthritis
  - Heart transplant

- Concerns about *responsiveness* to clinical change after intervention
  - Instrument not sensitive?
  - Intervention not effective?
- CHQ did not adequately reflect issues *unique* to the EOS population

Early Onset Scoliosis Questionnaire (EOSQ)

Purpose:

- To develop a disease specific instrument which reflects issues of importance to patients with EOS and caretakers, and is responsive to clinical changes after treatments


Literature Review
Existing Instruments
Expert Opinion
Semi-structured Interview Topic Items
Parent interviews
Mater List of 75 Items
Content Validity
Construct Validity
EOSQ
MS-CHONY
M.G. Vitale MD MPH

Master List of 75 Items

75 items in 11 Domains were produced:

1. General Health	6. Daily Living
2. Pain Discomfort	7. Pain
3. Pulmonary Function	8. Fatigue, Child Emotion
4. Physical Function	9. Surgical Burden
5. Transfer	10. Parental Burden
	11. Financial Burden

Item Statistics

E.g. Activity of Daily Living Domain

Item #	Floor	Ceiling	Mean	Normal Distribution	Item Reliability	Relevance & Clarity
33	19.2%	38.5%	3.31		.667 (vs. 34) .871 (vs. 35) .845 (vs. 36) .579 (vs. 37)	3.64
34	3.7%	81.5%	4.56		.667 (vs. 33) .795 (vs. 35) .677 (vs. 36) .767 (vs. 37)	3.35
35	3.7%	70.4%	4.41		.871 (vs. 33) .795 (vs. 34) .929 (vs. 36) .538 (vs. 37)	4.00
36	26.7%	53.3%	3.60		.845 (vs. 33) .677 (vs. 34) .929(vs. 35) .388 (vs. 37)	3.87
37	3.6%	82.1%	4.61		.579 (vs. 33) .767 (vs. 34) .538 (vs. 35) .388 (vs. 36)	4.30

Measuring Quality of Life in Children With Early Onset Scoliosis: Development and Initial Validation of the Early Onset Scoliosis Questionnaire

Jacqueline Corona, MD,† Hiroko Matsumoto, MA,*†*
David P. Roye, Jr, MD,† and Michael G. Vitale, MD, MPH*†*

Caregiver form with 24 items, 3 domains, 11 sub-domains

Raw scores transformed to 0-100 scale scores

Domain	Sub-Domain
Patient Quality of Life	General Health
	Pain/Discomfort
	Pulmonary Function
	Physical Function/Transfer
	Daily Living
	Fatigue/Energy Level
	Emotion
Family Burden	Parental Burden
	Financial Burden
Satisfaction	Patient Satisfaction
	Parent Satisfaction

EOSQ Project Overview

Phase 1 – Development of the EOSQ
COMPLETE

Phase 2 - Initial Validation and Responsiveness
COMPLETE

Phase 3 – Collection of Age-Based Normative Data
COMPLETE

Phase 4 - Prospective Validation, Reliability
COMPLETE

Phase 5 – Cross Translation
Translation of the EOSQ

English	PUBLISHED
Spanish	Dr. Farrington – PUBLISHED
Hispanic Spanish	Dr. Flynn & Dr. Ramirez – PUBLISHED
Turkish	Dr. Yazici - PUBLISHED
Danish	Dr. Bünger - translation underway
Japanese	Dr. Kawakami - translation underway
Mandarin Chinese	Dr. Xu - translation underway
French	Translation underway at McGill Univ.

Validation, Reliability and Responsiveness

Validity: Does it measure what it says it measures ?

- Content
- Construct
- Criterion

Reliability: Does it measure consistently ?

- internally consistent
- test- retest
  - (Cronbachs Alpha)

Responsiveness: in an expected manner to clinical change

Validity

Cohort	Domain	Pre-op	Age Norm
Idiopathic	Physical Function	**94**	97
	Fatigue	**88**	92
	Emotion	**81**	95

Idiopathic pts with EOS have small differences in QOl c/w Norms

Validity

Cohort	Domain	Pre-op	Age Norm
SMA	Pulmonary Function	**58**	98
	Transfer	**39**	99
	Fatigue	**41**	93
	Emotion	**53**	94
	Parental Burden	**40**	93

Patients with Other Comorbidities have much more significant decreases in QOL

Multiple EOSQ-24 Domain Scores Positively Correlated With % Predicted Values of FVC and FEV1

	Percent FEV1 Predicted	Percent FVC Predicted	Percent FEV1/FVC Predicted
EOSQ Domain	r value	r value	r value
General Health	0.78*	0.78*	-0.05
Pain/Discomfort	0.68*	0.58	0.27
Pulmonary Function	0.72*	0.61	0.34
Transfer	0.36	0.39	-0.10
Physical Function	0.69	0.74*	-0.16
Daily Living	0.69*	0.71*	-0.11
Faitgue/Energy Level	0.78**	0.80**	-0.03
Emotion	0.82**	0.79*	0.05
Parental Burden	0.56	0.45	0.16
Financial Burden	0.08	0.14	-0.32
Child Satisfaction	0.47	0.43	0.25
Parental Satisfation	0.28	0.22	0.37
N = 10	* = p < .05, ** = p < 0.01		

Complications worsen Pain; Without complication, Pulmonary Function and Parental Burden Improve

Cohort	Domain	Preop	Postop 1st/2nd	P	Age Norm
With Intra-op Complication	Pain	**72**	**59**	0.092	95
No Post-op Complication	Pulmonary Function	**83**	**88**	0.075	97
	Parental Burden	**61**	**66**	0.056	91

Test-Retest *Reliability* is Strong (>0.7) for all Domains Except Emotion.

EOSQ Domain	Correlation Coefficient
General Health	.84**
Pain/Discomfort	.85**
Pulmonary Function	.90**
Transfer	.84**
Physical Function	.97**
Daily Living	.98**
Faitgue/Energy Level	.92**
Emotion	.68*
Parental Burden	.80*
Financial Burden	.94**
Child Satisfaction	.93**
Parental Satisfation	0.89**
N = 15	* = $p < .05$, ** = $p \leq 0.01$

Responsiveness

Cohort	Domain	Pre-op	Post-op	P	Age Norm
SMA	Pulmonary Function	**58**	**98**	0.083	98
	Transfer	**39**	**61**	0.045	99
	Fatigue	**41**	**52**	0.078	93
	Emotion	**53**	**69**	0.080	94
	Parental Burden	**40**	**63**	0.008	93

SMA pts show EOSQ improvements after Growing Rods/ VEPTR

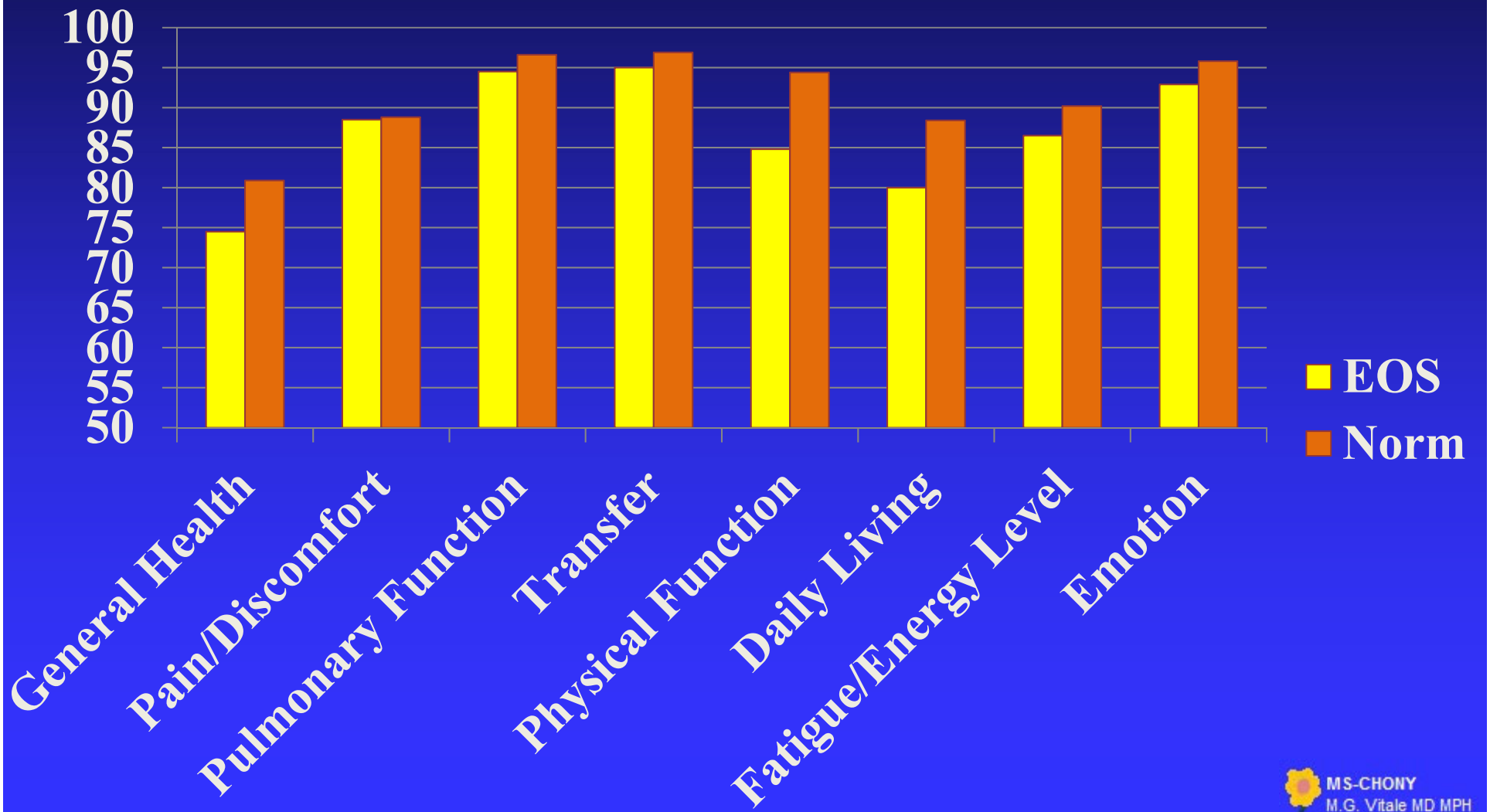
Responsiveness

Cohort	Domain	Pre-op	Post-op	P	Age Norm
Idiopathic	Physical Function	**94**	**84**	0.048	97
	Fatigue	**88**	**69**	0.076	92
	Emotion	**81**	**60**	0.006	95

Idiopathic pts, worsened in Physical Function, Fatigue, and Emotion

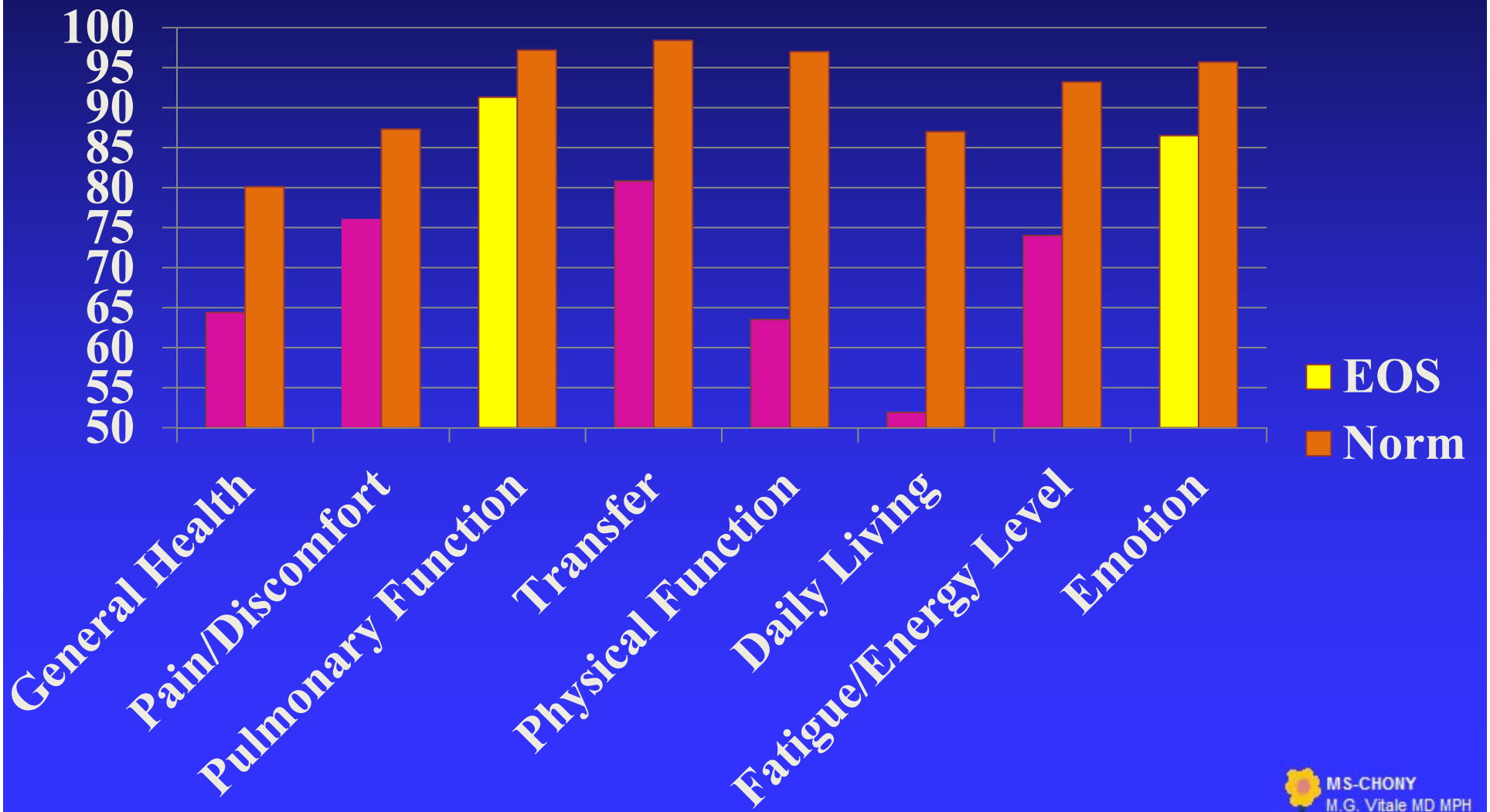
Idiopathic: Pre-Casting

No significant difference in HRQoL compared to norms



Non-Idiopathic: Pre-Casting

Some HRQoL significantly lower compared to norms



Transfer, Physical Function, Emotion, and Child Satisfaction All Worsened from Pre- to Post-Casting Visits

Pre-Casting vs Post-Casting (N = 15)			
Domain Name	Mean ± SD		p
	EOS	Norm	
General Health	75 ± 23	73 ± 20	0.700
Pain/Discomfort	89 ± 18	86 ± 19	0.573
Pulmonary Function	95 ± 11	87 ± 22	0.173
Transfer	100 ± 0	83 ± 20	0.007
Physical Function	88 ± 19	65 ± 23	0.010
Daily Living	74 ± 33	57 ± 27	0.073
Fatigue/Energy Level	89 ± 13	76 ± 32	0.170
Emotion	97 ± 7	88 ± 14	0.054
Parental Burden	80 ± 21	72 ± 24	0.257
Financial Burden	83 ± 20	72 ± 27	0.110
Child Satisfaction	90 ± 13	69 ± 29	0.020
Parent Satisfaction	85 ± 21	70 ± 29	0.108

Responsiveness to Clinical Change

Cohort	Domain	Pre-op	Post-op 1st/2nd	P	Age Norm
SMA	Pulmonary Function	**58**	**98**	0.083	98
	Transfer	**39**	**61**	0.045	99
	Fatigue	**41**	**52**	0.078	93
	Emotion	**53**	**69**	0.08	94
	Parental Burden	**40**	**63**	0.008	93

EOSQ Reflects Health Status Improvement in SMA pts after Growing Rods

EOSQ as Primary Endpoint

Do externally controlled growth rods lead to better HRQL outcomes?

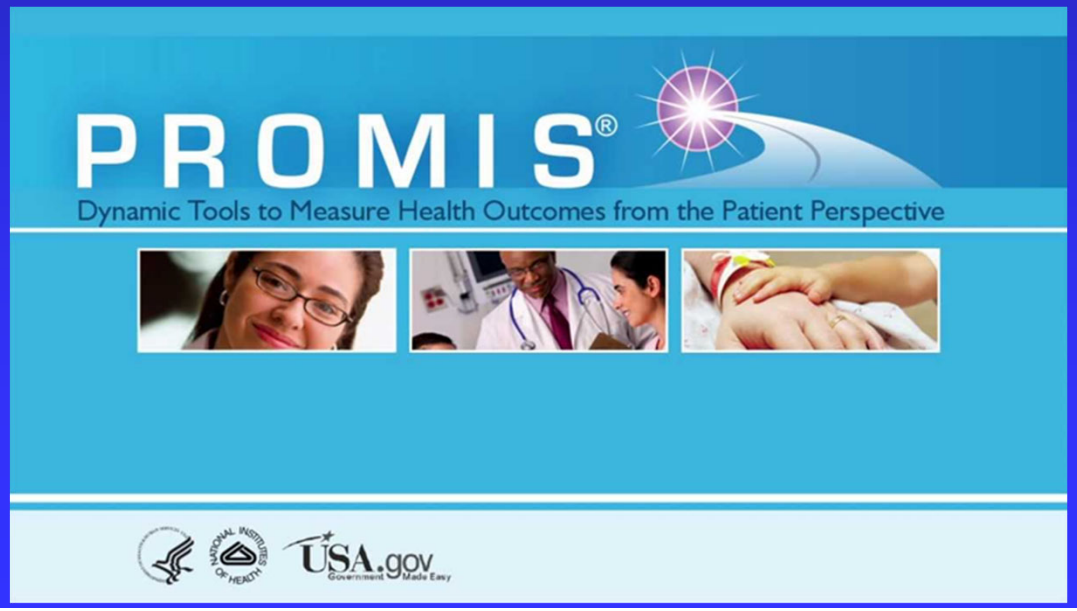
Prospective study of TGR vs MAGEC with EOSQ as primary endpoint

Conclusion:
What Have We Learned from the EOSQ ?

- Patients with EOS have lower QOL, even the idiopathic patients
- Intervention seems to improve QOL in nonidiopathic but worsen QOL in idiopathic
- Complications decrease QOL
- Casting Effects Qol in Children
- EOSQ is valid, relaibale and responsive and can be used as a PRO measure for EOS

Final Thoughts: What about PROMIS ?

- PROMIS: Patient-Reported Outcomes Measurement Information System.
- **NIH-funded** psychometric evaluation that has grown significantly over last several years: (http://www.hhs.gov/asl/testify/t051208a.html)



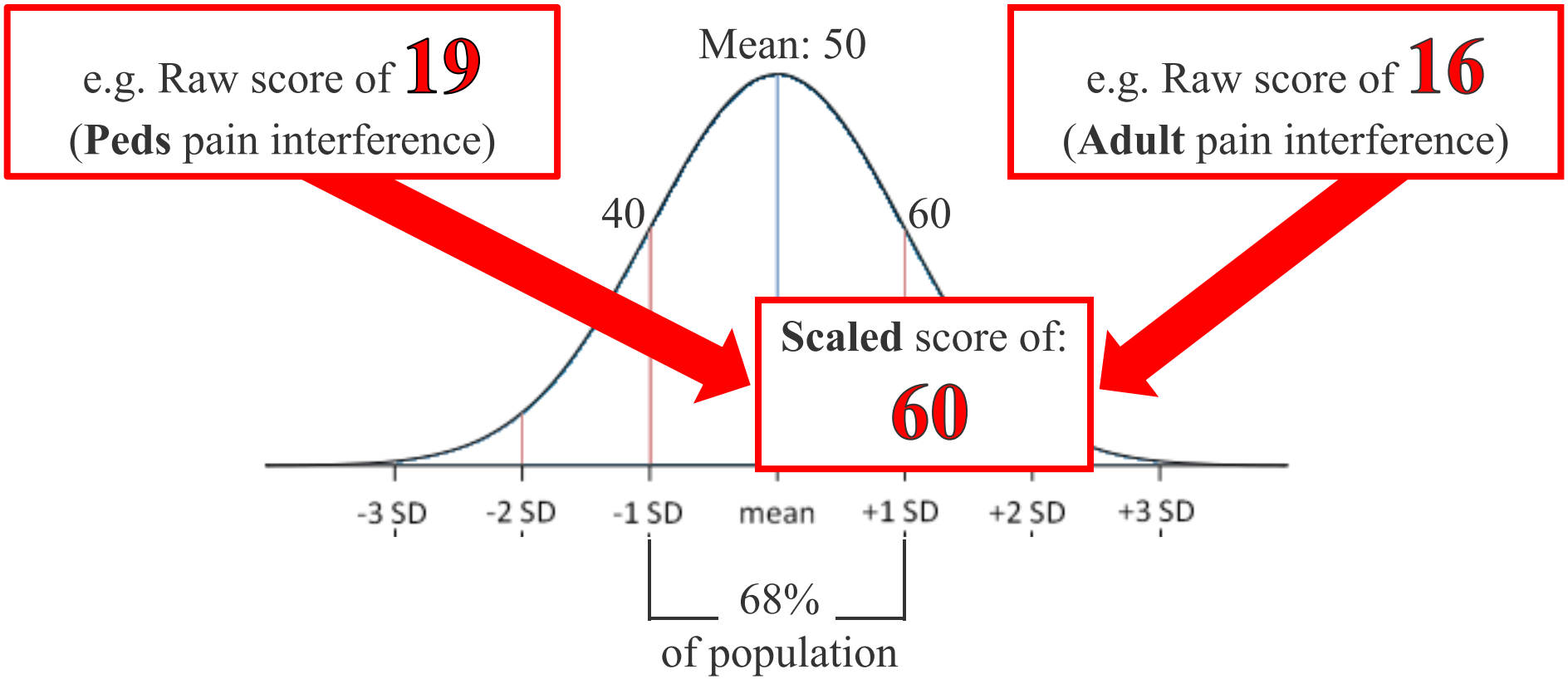
PROMIS is dynamic

CAT: Computer Adaptive Test

- Dynamic option: Questions are presented in changing sequence, with following sequence based on prior answers.
- Purpose:
  - Minimizes # questions;
  - Greater measurement precision;

PROMIS

- One of the biggest advantages of the PROMIS scaled scores is that they have been adjusted so that **adult and pediatric scores can be compared directly.**



PROMIS has Infrastructure

- Study specific instruments by choosing domains of your interest
- Assessment Center (www.assessmentcenter.net)
  - A free online data collection tool for PROMIS
- Patients answer questions by logging into the website
- Scaled scores are calibrated so that: (NIHpromis.org/faqs)
  - A score of **50-points** is the average score of the US population;
  - **10 -points** is equal to one standard deviation.

MORE TO COME ….

Thank You

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