# Using Pediatric PROMIS (Patient Reported Outcomes Measurement Information System) to Evaluate Symptom Burden Experienced by Children with Brain Tumors

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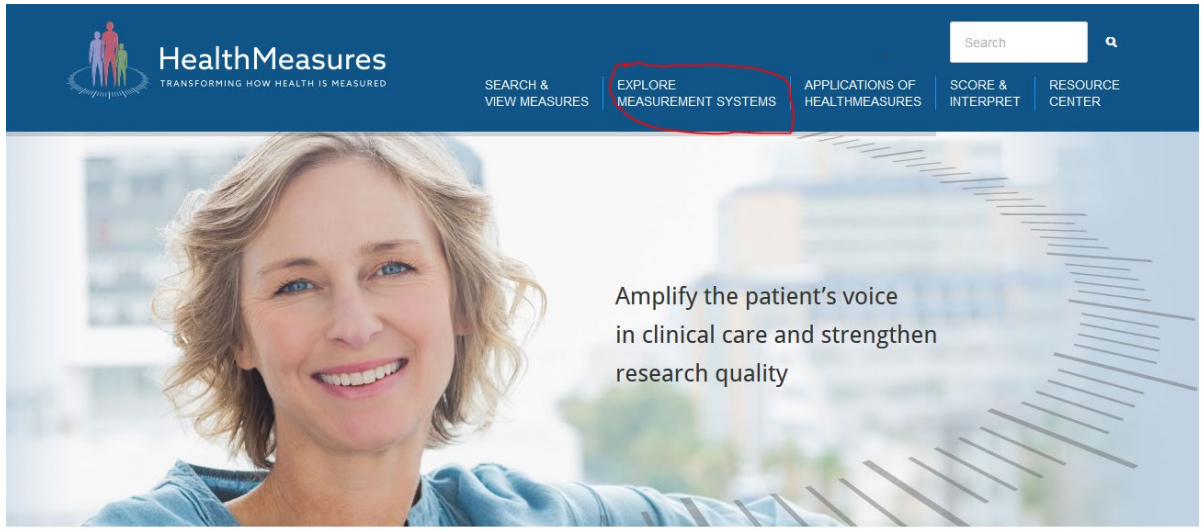
# What is PROMIS®?

**Patient-Reported Outcomes Measurement Information System®**

- Supported by the NIH Blueprint/Common Fund (2004-2018)
- Now supported by HealthMeasures, the official information & distribution center for 4 NIH-supported measurement systems (PROMIS®, Neuro-QoL, NIH Toolbox®, & ASCQ-Me®)




HealthMeasures consists of four precise, flexible, and comprehensive measurement systems that assess physical, mental, and social health, symptoms, well-being and life satisfaction; along with sensory, motor, and cognitive function.

[HealthMeasures.net/PROMIS](HealthMeasures.net/PROMIS)

PROMIS®

HealthMeasures
TRANSFORMING HOW HEALTH IS MEASURED

# What is PROMIS®?

- Measures used to evaluate and monitor physical, mental, and social health (adult & pediatric)
- More than 50 research protocols aligned with evolving PROMIS standards
- More than 60,000 people contributed data
  - Adults: **1,402 questions** populating >80 “item banks”
  - Children: **462 questions** populating >20 item banks
- English, Spanish, Dutch, German, and other languages emerging
- Universal concepts; expandable to specific issues
- International PROMIS working group
- Began as a US NIH effort to standardize patient reported outcomes for clinical research
- Expanded to clinical practice, quality measurement, population health, and international adoption

# Essential Components of PROMIS

## DOMAIN

The feeling, function, or perception you wish to measure

Cuts across different diseases and settings. E.g., physical function, depressive symptoms

## ITEM BANK

Collection of items that each measure the same domain

Used to create different measure types, all producing a score on the same metric

***Cuts across different diseases and facilities – much needed for patients with rare diseases such as children with brain tumors***

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# Children with Brain Tumors

- Children with a brain tumor are vulnerable to experiencing moderate or severe adverse events, which lead to poor health-related quality of life.
- It is challenging to evaluate comparative effectiveness with this population because
  - Brain tumors are both uncommon and diverse.
  - The functional impact of brain tumors and the range of surgical and treatment effects can vary based on characteristics of tumors such as location, size, and type.
- By focusing on common HRQOL domains, PROMIS offers an opportunity to address this deficit.
  - developed using item response theory models, which enables computerized adaptive tests (CATs)
  - scores are reported using T-score metric centered on the norming sample.

*Study 1: Assess HRQOL of children with brain tumor using the PROMIS measures of Anxiety, Depression, Fatigue, Mobility, Upper extremity function, Peer relationships and Cognition.*

Lai et al (2019). 14:e27526. doi: 10.1002/pbc.27526. PMID: 30426667

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# Sample

- N=230; mean age was 14.1 (SD=3.7; 7-22 years); 52% male; 76% white; 84% were newly diagnosed.
- 95% attending school
  - 49% mainstream classroom without receiving any form of individualized educational programs (e.g., special education classroom within a regular school or special education school)
- Average years since diagnosis was 4.1 (SD=4.5)
  - The most common histology was astrocytic tumors (grades 1-4; 28.3%), followed by medulloblastoma (20%) and glial tumors (12.7%);
  - 21.7% had one or more lesions in the posterior fossa, 10.9% in the thalamus and 10.4% in the brain stem.
- Average years since last treatment was 2.6 (SD=3.4);
  - 73.8% received surgery, 74.1% chemotherapy, 56.8% radiation (41.9% received proton therapy), and 34.1% received all three modes of therapy.

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# Measures

- Child
  - PROMIS (computerized adaptive testing, CAT): Anxiety, Depression, Fatigue, Mobility, Upper extremity function, Peer relationships
  - 10-item PROMIS Cognition short-form (a.k.a., pediatric perceived cognitive function item bank, pedsPCF).
  - Symptom Distress Scale
    - Getting around, tired, feeling miserable , sleep, appetite & cognition
- Parent
  - Proxy versions

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# Results – versus SDS

| | SDS - Physical | | SDS - Fatigue | | SDS - Emotion | | SDS - Sleep | | SDS - Appetite | | SDS - Cognition | |
|---|---|---|---|---|---|---|---|---|---|---|---|---|
| | F-value | p | F-value | p | F-value | p | F-value | p | F-value | p | F-value | p |
| **Compared to the Child-rated Symptom Distress Scale (SDS)** | | | | | | | | | | | | |
| **Anxiety** | 9.91 | <.0001 | 9.93 | <.0001 | 8.78 | <.0001 | 3.69 | 0.0132 | 3.98 | 0.0091 | 9.71 | <.0001 |
| **Depression** | 11.14 | <.0001 | 9.45 | <.0001 | 9.08 | <.0001 | 5.83 | 0.0008 | 4.53 | 0.0044 | 7.93 | <.0001 |
| **Fatigue** | 20.38 | <.0001 | 25.00 | <.0001 | 8.13 | <.0001 | 11.94 | <.0001 | 16.37 | <.0001 | 23.32 | <.0001 |
| **Mobility** | 23.89 | <.0001 | 7.33 | 0.0001 | 4.53 | 0.0043 | 5.15 | 0.0019 | 8.05 | <.0001 | 9.28 | <.0001 |
| **Upper Extremity** | 10.71 | <.0001 | 5.17 | 0.0019 | 1.88 | 0.1348 | 0.89 | 0.4469 | 5.12 | 0.0020 | 3.27 | 0.0226 |
| **Peer Relationships** | 1.91 | 0.1295 | 5.13 | 0.0021 | 3.25 | 0.0235 | 1.27 | 0.2864 | 2.40 | 0.0694 | 5.10 | 0.0022 |
| **Cognition** | 4.81 | 0.0030 | 12.73 | <.0001 | 4.65 | 0.0037 | 7.09 | 0.0002 | 11.74 | <.0001 | 15.70 | <.0001 |
| **Compared to the Parent rated Symptom Distress Scale (SDS)** | | | | | | | | | | | | |
| **Anxiety** | 1.52 | 0.2121 | 5.71 | 0.0010 | 8.07 | <.0001 | 1.91 | 0.1303 | 2.77 | 0.0441 | 9.52 | <.0001 |
| **Depression** | 3.85 | 0.0110 | 10.25 | <.0001 | 14.16 | <.0001 | 3.23 | 0.0245 | 10.52 | <.0001 | 11.01 | <.0001 |
| **Fatigue** | 8.27 | <.0001 | 16.29 | <.0001 | 11.04 | <.0001 | 4.58 | 0.0042 | 13.17 | <.0001 | 12.72 | <.0001 |
| **Mobility** | 16.06 | <.0001 | 6.20 | 0.0005 | 5.23 | 0.0018 | 1.67 | 0.1757 | 4.34 | 0.0057 | 5.18 | 0.0019 |
| **Upper Extremity** | 16.18 | <.0001 | 4.84 | 0.0031 | 4.25 | 0.0065 | 0.63 | 0.5998 | 3.04 | 0.0309 | 3.50 | 0.0171 |
| **Peer Relationships** | 1.91 | 0.1310 | 2.43 | 0.0679 | 4.98 | 0.0026 | 5.03 | 0.0025 | 1.13 | 0.3386 | 2.39 | 0.0720 |
| **Cognition** | 2.12 | 0.0994 | 6.38 | 0.0004 | 3.28 | 0.0225 | 3.45 | 0.0180 | 3.75 | 0.0122 | 12.50 | <.0001 |

Planned analyses

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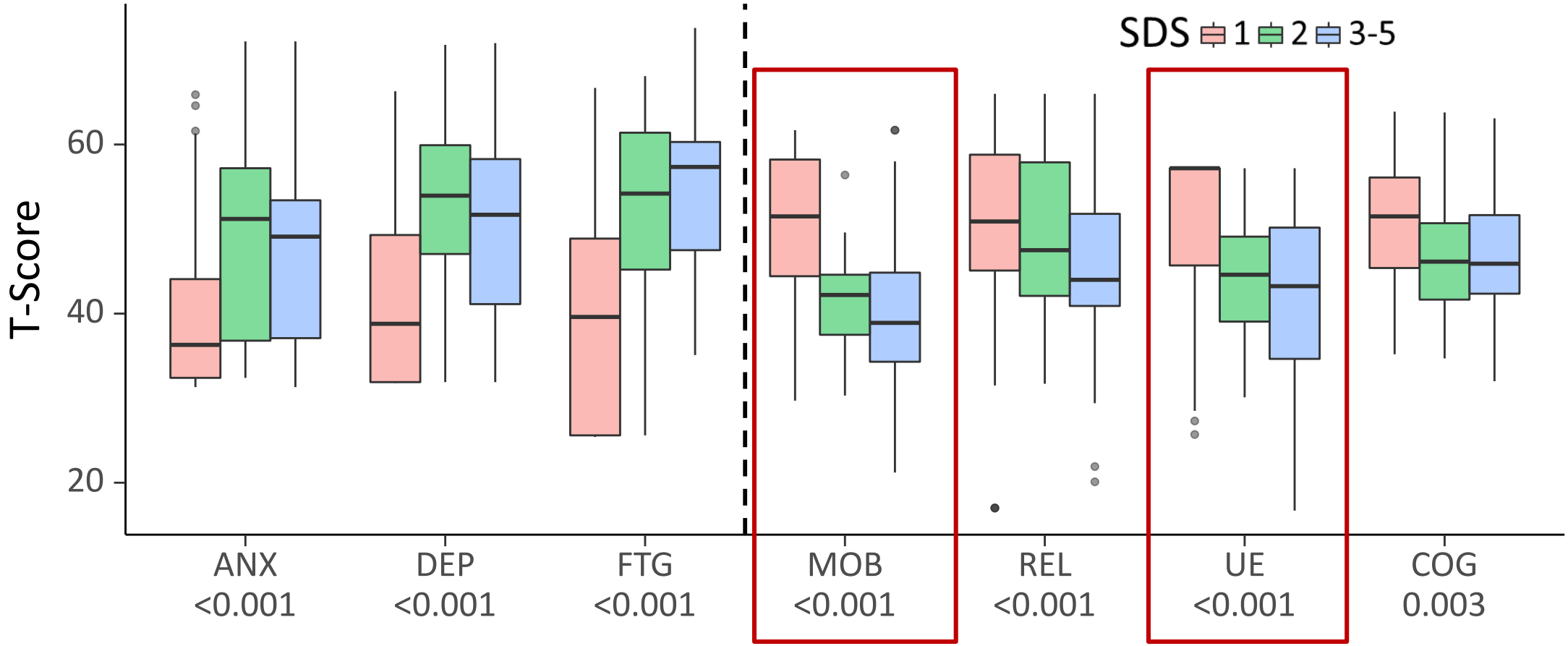
# *Specifically. . . . versus SDS-Physical:*




*SDS: 1=least distress, 5=worst distress*

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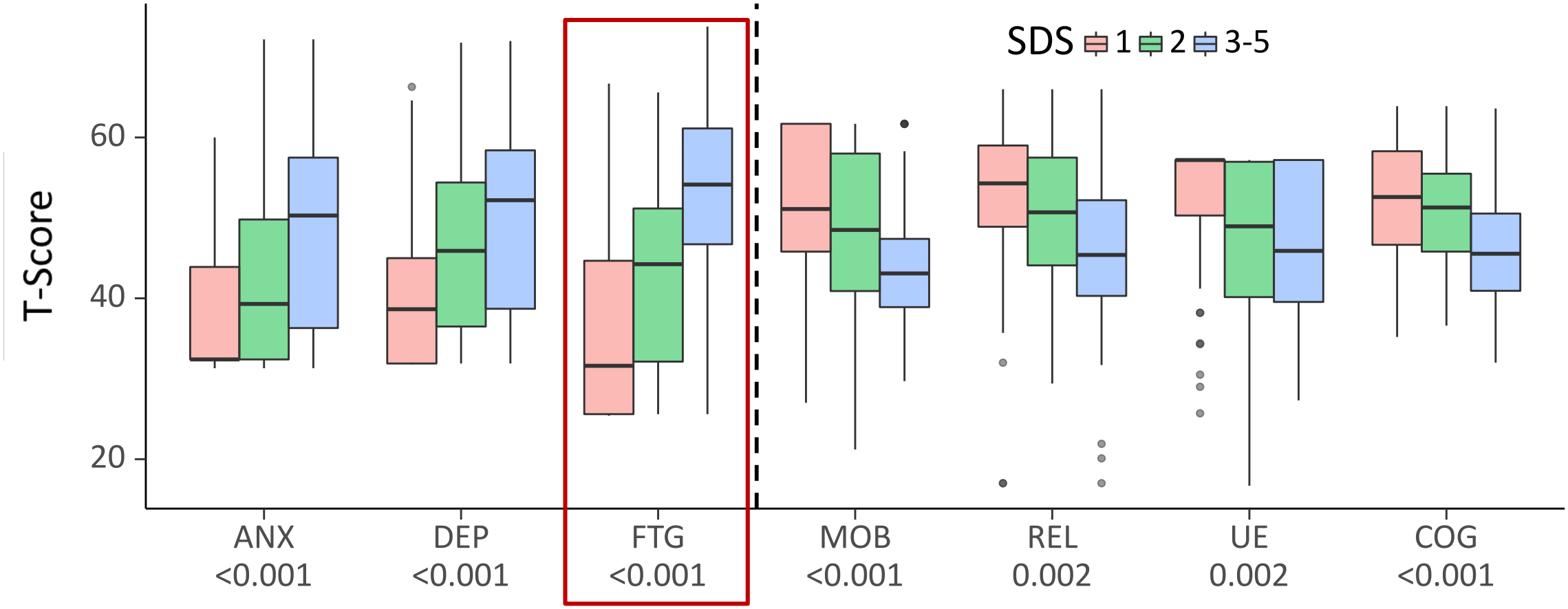
# *Specifically. . . . versus SDS-Fatigue:*




*SDS: 1=least distress, 5=worst distress*

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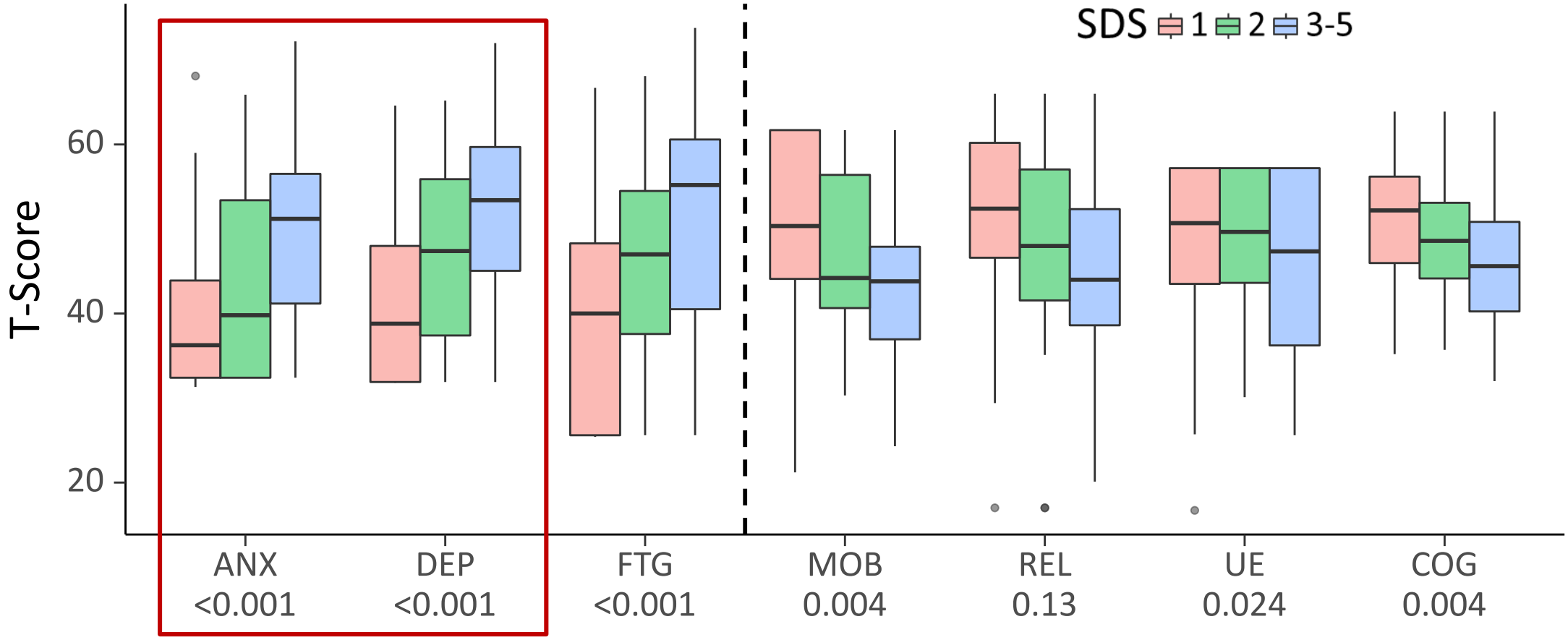
# *Specifically. . . . versus SDS-Emotion:*




*SDS: 1=least distress, 5=worst distress*

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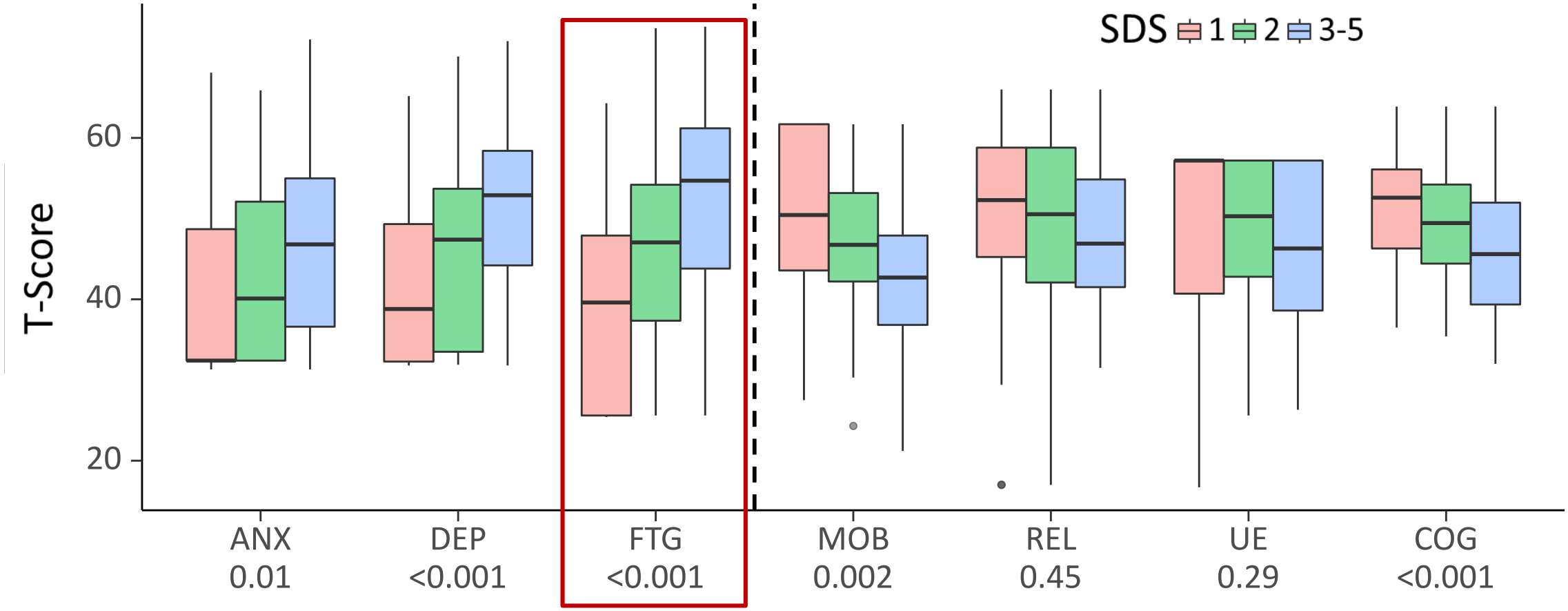
# *Specifically. . . . versus SDS-Sleep:*




*SDS: 1=least distress, 5=worst distress*

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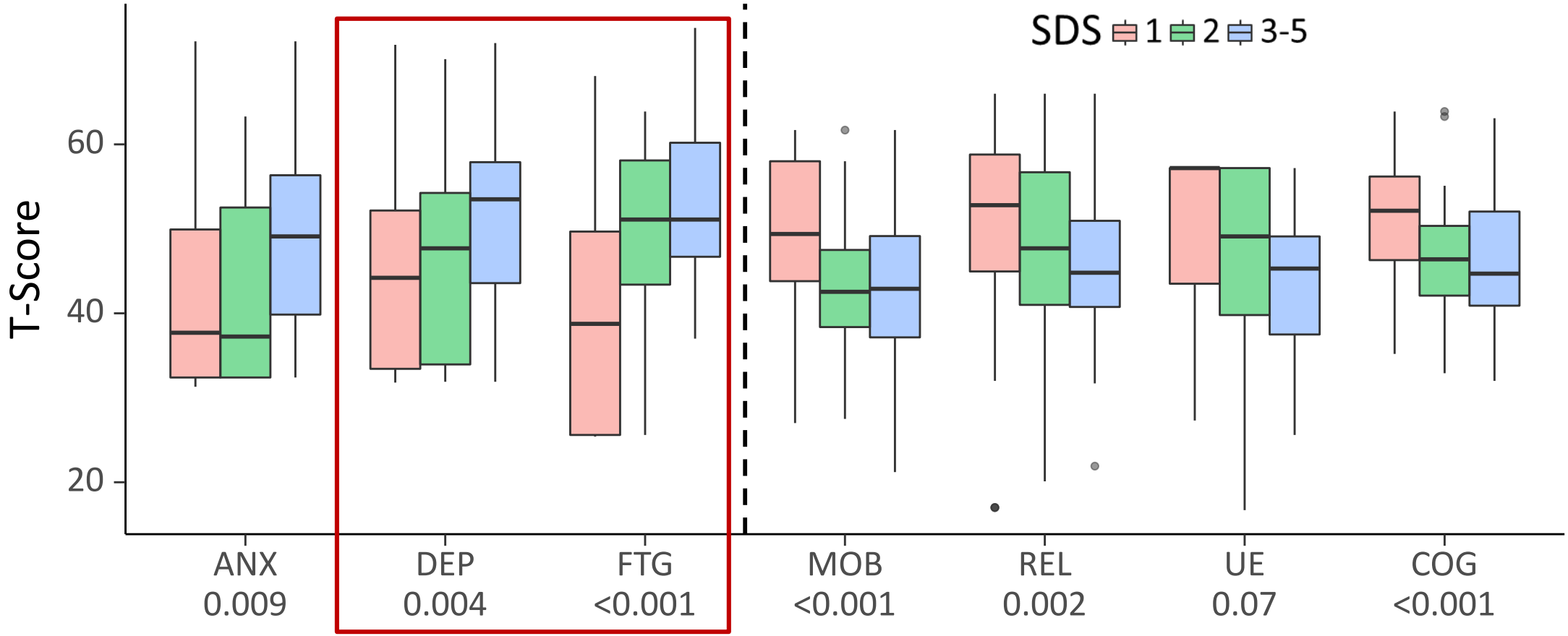
# *Specifically. . . . versus SDS-Appetite:*




*SDS: 1=least distress, 5=worst distress*

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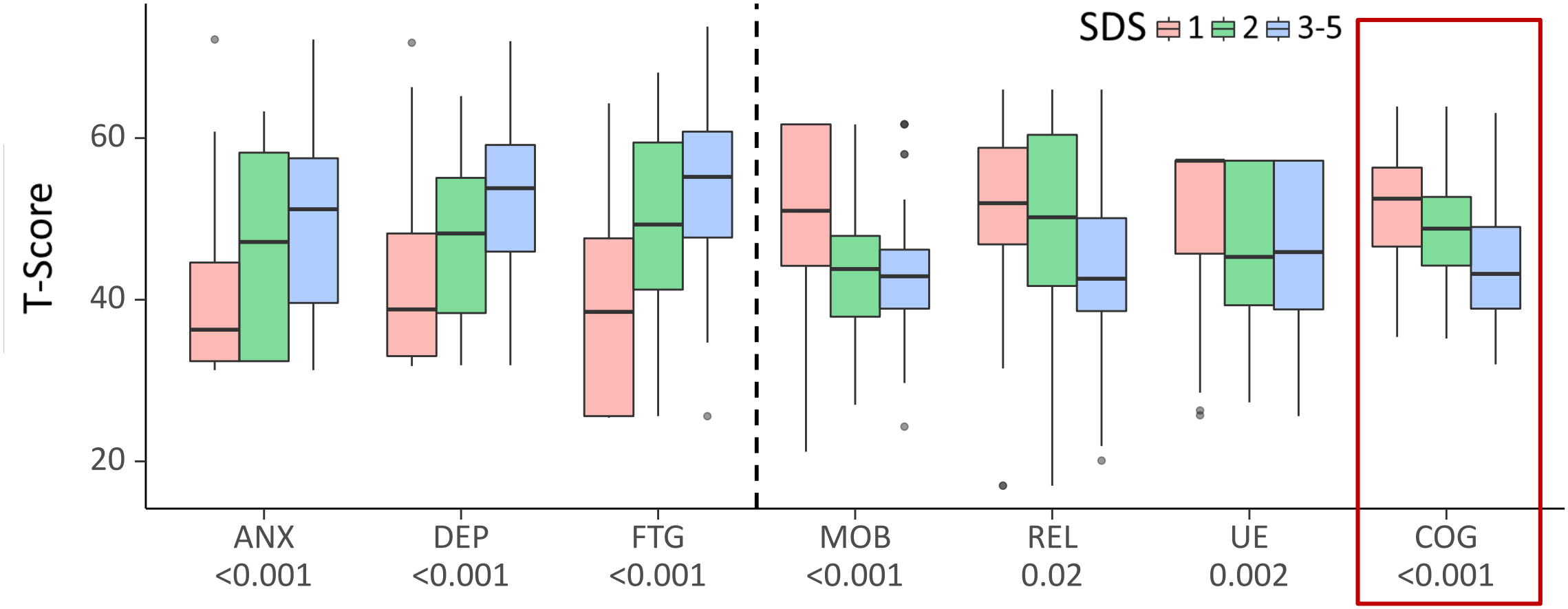
# *Specifically. . . . versus SDS-Cognition:*




*SDS: 1=least distress, 5=worst distress*

# PROMIS vs. clinical variables, educational programs and parent-rated child's HRQOL

| | Parent-rated QOL | | Karnofsky Performance Rating | | Educational Program [a] | | Time since last radiation [b] | | Time since last chemotherapy [b] | | Treatment modalities & time since last treatment [b] | |
|---|---|---|---|---|---|---|---|---|---|---|---|---|
| | F | p | F | p | F | p | F | p | F | p | F | p |
| ***Higher scores represents worse symptomatic*** | | | | | | | | | | | | |
| Anxiety | 4.83 | 0.001** | 1.98 | 0.142 | 0.17 | 0.681 | 0.31 | 0.733 | 4.18 | 0.017* | 0.91 | 0.457 |
| Depression | 8.04 | <0.0001** | 1.38 | 0.254 | 0.34 | 0.563 | 1.46 | 0.235 | 2.05 | 0.132 | 1.52 | 0.199 |
| Fatigue | 14.29 | <0.0001** | 5.43 | 0.005** | 4.6 | 0.034* | 2.73 | 0.068 | 1.3 | 0.274 | 3.03 | 0.019* |
| ***Higher scores represents better functioning*** | | | | | | | | | | | | |
| Mobility | 8.15 | <0.0001** | 18.99 | <.0001** | 3.12 | 0.079 | 4.44 | 0.013* | 3.38 | 0.036* | 3.26 | 0.013* |
| Upper Extremity Function | 4.11 | 0.004** | 14.4 | <.0001** | 3.38 | 0.068 | 4.89 | 0.009** | 6.45 | 0.002** | 3.4 | 0.011* |
| Peer Relationships | 4.17 | 0.003** | 0.87 | 0.422 | 1.44 | 0.233 | 1.92 | 0.150 | 2.53 | 0.083 | 1.78 | 0.136 |
| Cognition | 6.35 | <0.0001** | 2.61 | 0.077 | 25.29 | <.0001** | 0.04 | 0.957 | 0.69 | 0.505 | 0.36 | 0.838 |

a. "Regular classroom w/o any forms of IEP" vs. "received any forms of IEP or special education"

b. "never received tx" vs. within 1 year" vs. "> 1 year"

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# Conclusions

- All PROMIS measures were significantly associated with Symptom Distress Scale reported by patients and parents
- Treatment, time since treatment, parent-rated QOL, educational program and performance ratings were associated with HRQOL
  - Domain dependent

# *Study 2:*

# *Using Pediatric PROMIS to Evaluate Changes of the Symptom Burden Over Time*

# Objectives

Monitoring symptom burden reported by patients and parents using pediatric PROMIS *Anxiety, Depressive Symptoms, Fatigue, Mobility, Upper Extremity Function*, and *Peer Relationships* CATs, and *Cognition* brief, fixed-form over 12 months.

- Patterns of PRO changes reported by patients and their parents as well as factors associated with these patterns.
- Correlation between patient- and parent-reported patient symptom burden.
- Whether symptom burden reported by patients and parents predicted patient survival rate.

*Symptom burden and HRQOL are used interchangeably*

# Sample (N=289)

- **Patient**
  - age: 12.4 yrs (range: 5-22; SD=4.7); 54.5% male; 78.6% white
  - Years since recent tx: 0.39 (SD=1.2)
- **Parent**
  - age: 43.0 yrs (SD=7.0); 17.4% male

| | | All patients (N=289) | Patients with vs. without 12-month Follow-up w/o (n=150) | With (n=139) | |
|---|---|---|---|---|---|
| Does your child go to school? | Yes | 93.0 | 90.8 | 96.0 | 0.098 |
| Type of classroom attending | Mainstream classroom, no IEP | 49.6 | 50.0 | 48.3 | 0.432 |
| | Mainstream classroom, with IEP | 35.3 | 35.6 | 35.6 | |
| | Special education classroom within a regular school | 7.1 | 5.9 | 8.5 | |
| | Special education school | 1.3 | 2.5 | 0.0 | |
| | Other | 6.7 | 5.9 | 7.6 | |
| Histology | Low grade glioma | 23.5 | 25.7 | 21.7 | 0.044 |
| | Medulloblastoma & other embryonal tumors | 22.8 | 18.9 | 26.8 | |
| | Glioneuronal tumor | 11.1 | 5.4 | 16.7 | |
| | Ependymoma | 7.3 | 6.8 | 8.0 | |
| | Germinoma | 6.9 | 6.1 | 7.3 | |
| | High grade glioma | 5.5 | 7.4 | 3.6 | |
| Current Status of Tumor | Initial diagnosis only | 86.3 | 81.5 | 91.3 | 0.017 |
| | Recurrent | 13.7 | 18.5 | 8.7 | |
| Treatments received | none | 4.5 | 4.1 | 5.1 | 0.222 |
| | 1 of 3 possible treatments | 24.2 | 27.7 | 19.6 | |
| | 2 of 3 possible treatments | 33.2 | 35.1 | 31.9 | |
| | Chemo+radiation+surgery | 38.1 | 33.1 | 43.5 | |
| Treatments | Radiation | | | | 0.018 |
| | No radiation | 39.5 | 39.2 | 39.9 | |
| | <=1 year | 29.4 | 35.8 | 22.5 | |
| | > 1 year | 31.1 | 25.0 | 37.7 | |
| | Chemotherapy (missing=6) | | | | <0.001 |
| | No chemotherapy | 25.5 | 34.7 | 15.4 | |
| | <=1 year | 37.8 | 38.1 | 37.5 | |
| | > 1 year | 36.7 | 27.2 | 47.1 | |
| | Surgery (missing n=5) | | | | 0.866 |
| | No surgery | 28.9 | 28.4 | 29.2 | |
| | <=1 year | 21.3 | 22.3 | 19.7 | |
| | > 1 year | 49.8 | 49.3 | 51.1 | |
| Type of radiation received | Photon | 44.7 | 51.1 | 38.8 | 0.271 |
| | Proton | 52.9 | 46.6 | 58.8 | |
| | Both photon and proton | 2.4 | 2.3 | 2.5 | |
| Years since last treatment | <= 1 year | 83.9 | 84.1 | 83.5 | 0.886 |
| | > 1 year | 16.1 | 15.9 | 16.5 | |

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# Results

- **Linear mixed-effects models** - symptom changes over time at the group level
  - Patient-reported Cognition (t=-2.11, p=0.037) & parent-reported Anxiety (t=2.18, p=0.0333) got significantly worse over time
- **Cox proportional hazards model** – survival analysis
  - 24 deaths
  - Patient-reported Mobility (hazard ratio=0.725, p=0.011) and Upper Extremity Function (HR =0.703, p=0.006) predicted better survival.
  - Longer time since diagnosis and higher performance rating were also predictive of survival.
- **Latent class growth analysis** (LCGA) to investigate patterns of symptoms change over time at the individual level. Numbers of classes within each domain
  - ranged 2 and 5 for patients;
  - ranged 2 and 3 for parents across domains.

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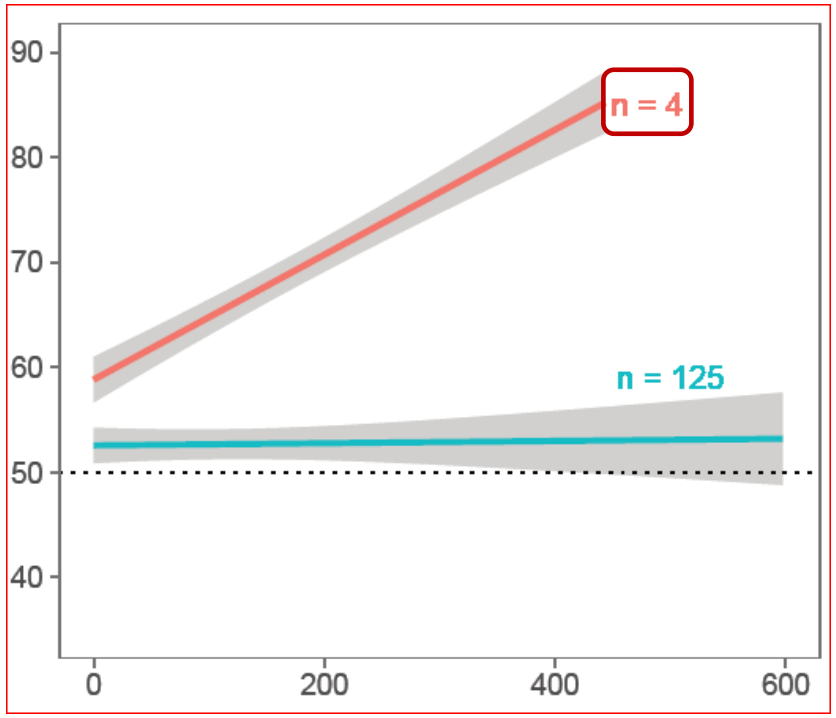
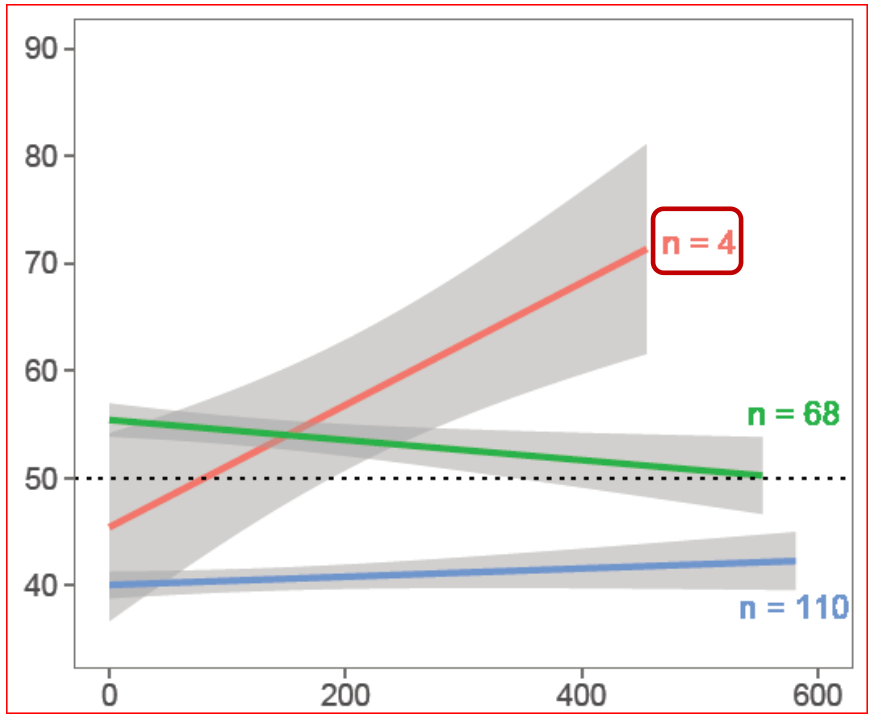
# Anxiety

## PARENT




## PATIENT




Correlation between change scores of parents and patients: r = 0.421, p=0.0819

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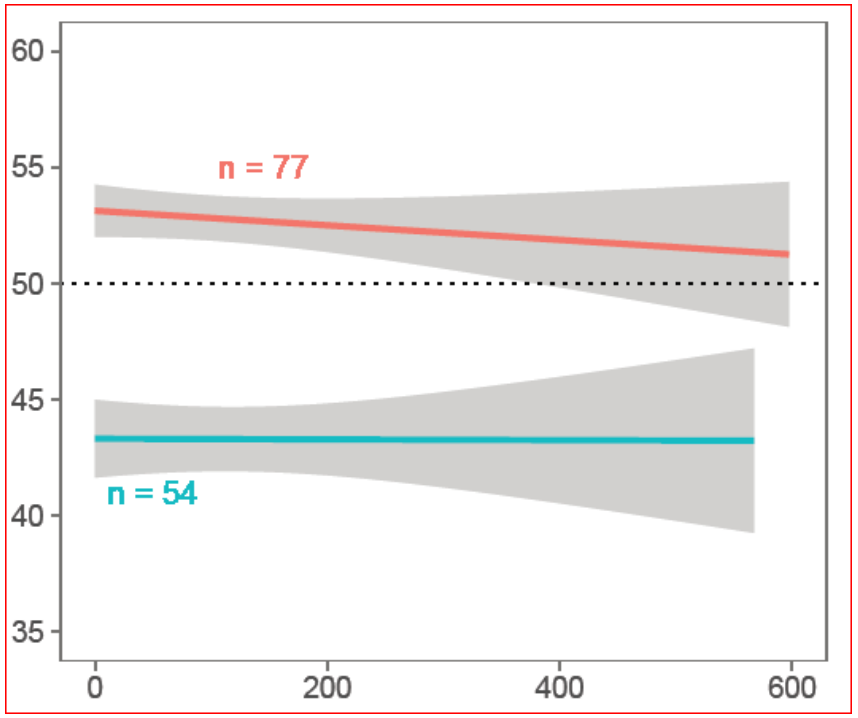
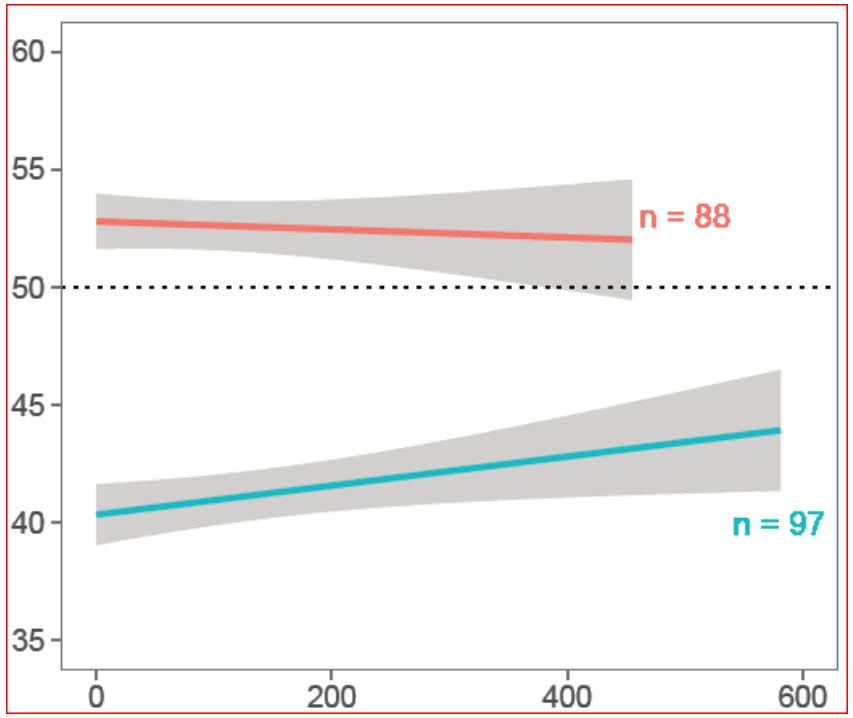
# Depressive Symptoms

**PARENT**




**PATIENT**




Correlation between change scores of parents and patients: r = 0.708, p=0.0005

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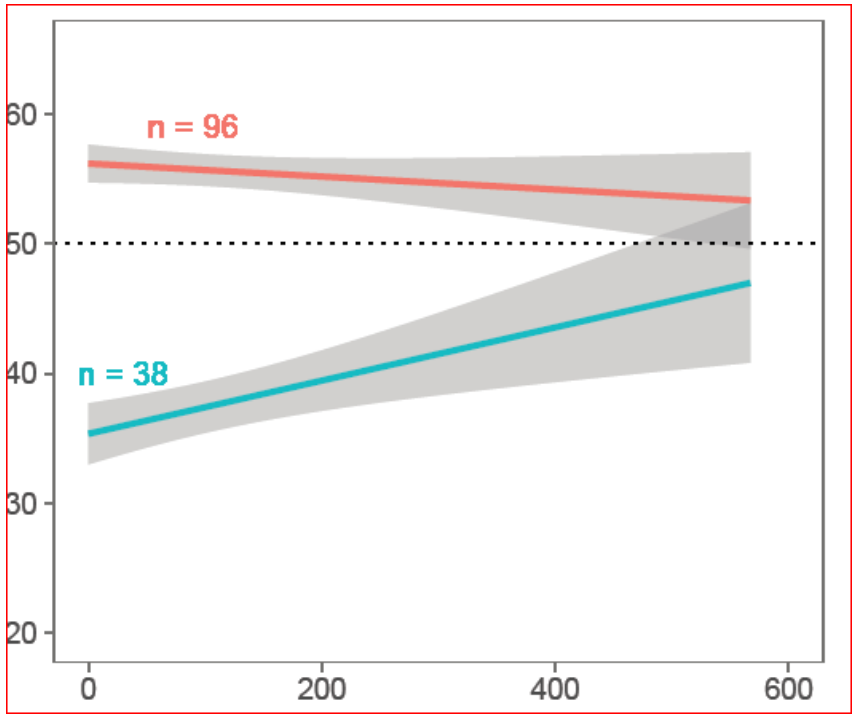
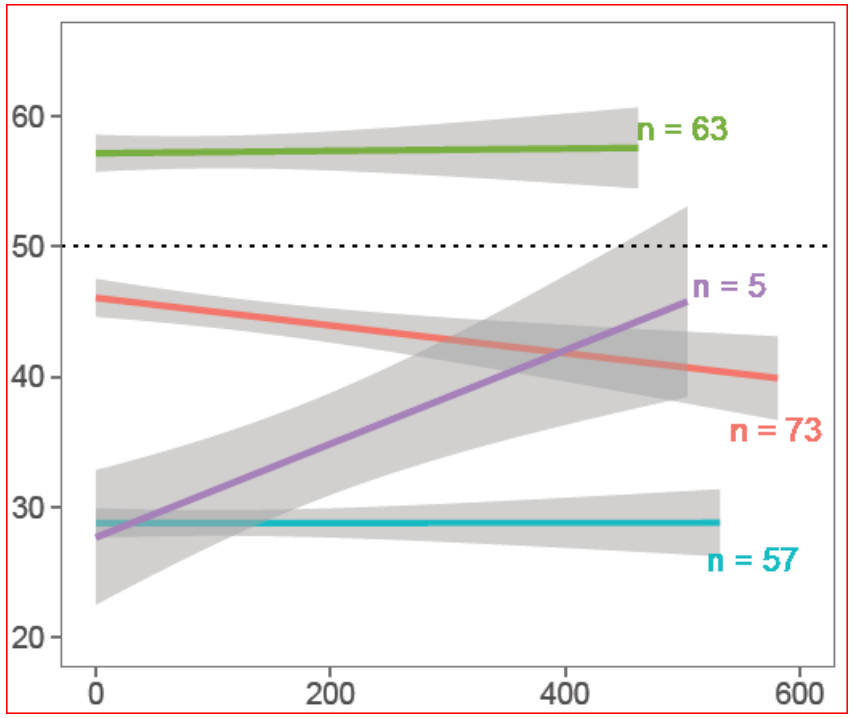
# Fatigue

**PARENT**




**PATIENT**




Correlation between change scores of parents and patients: r = 0.49, p=0.0150

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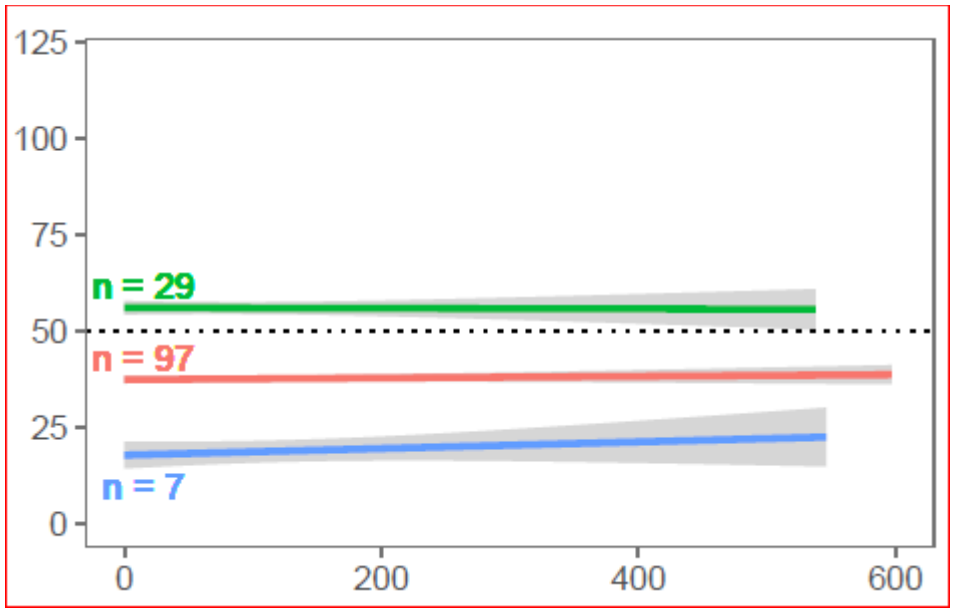
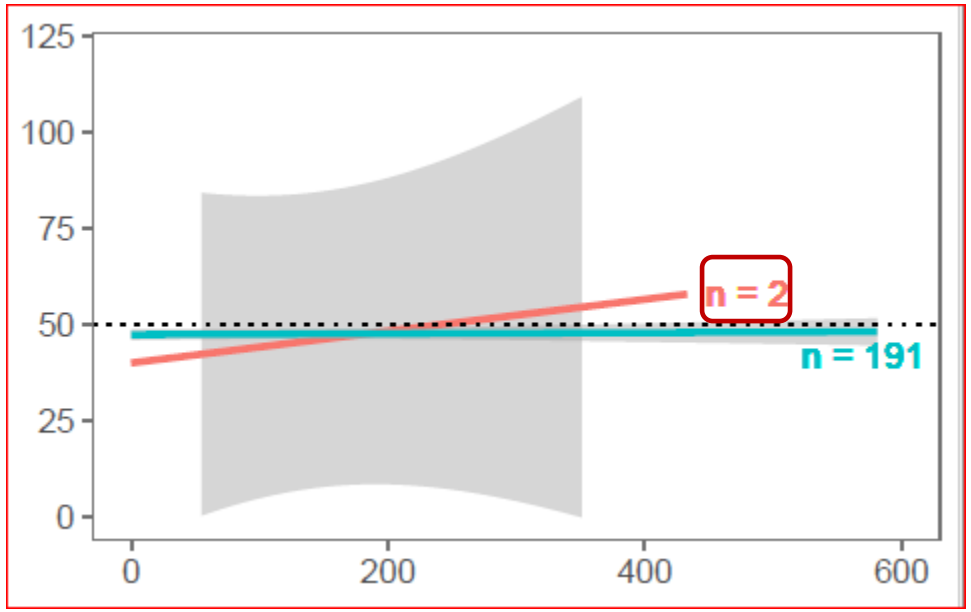
# Mobility

**PARENT**




**PATIENT**




Correlation between change scores of parents and patients: r = 0.44, p=0.0355

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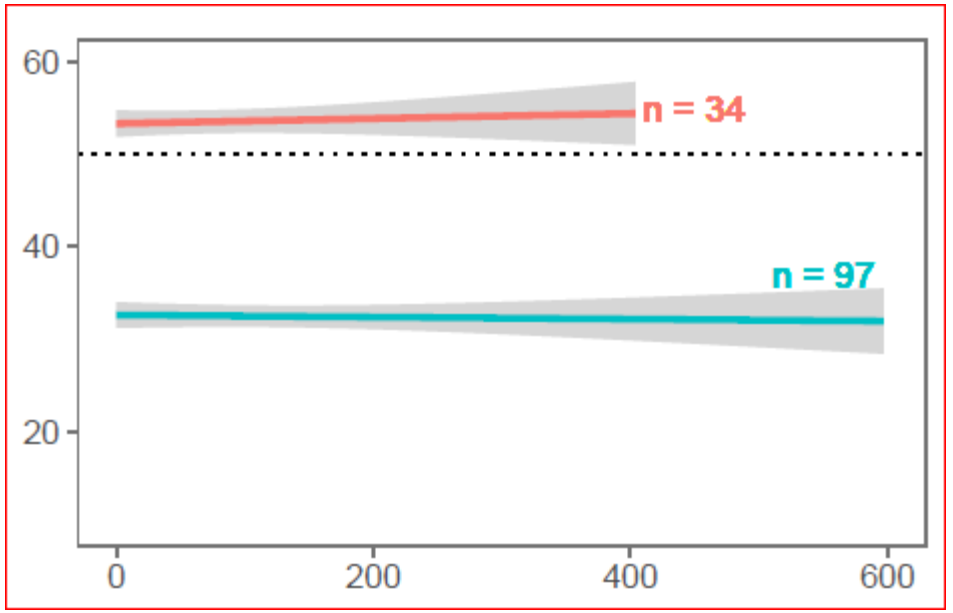
# Upper Extremity Function

**PARENT**

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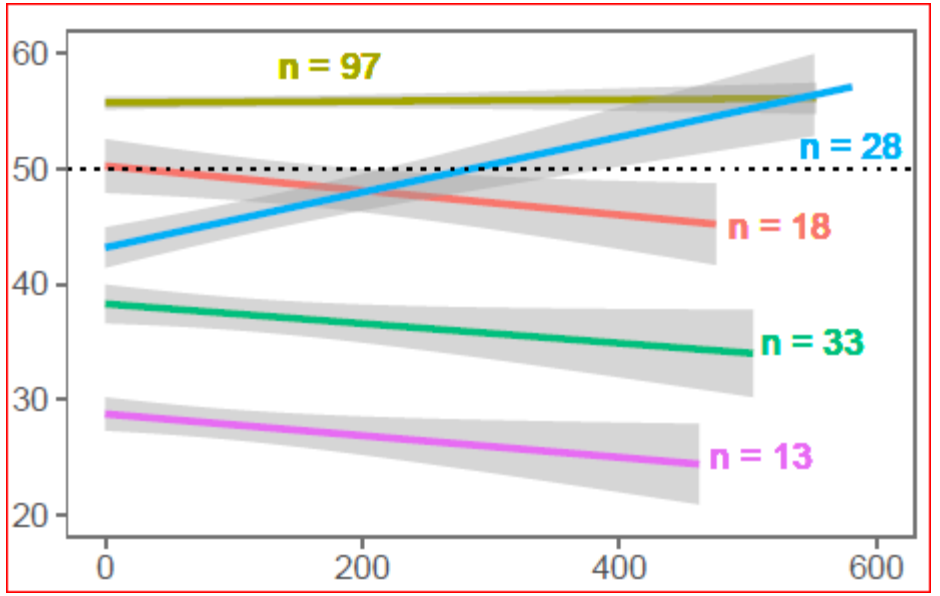


Correlation between change scores of parents and patients: r = 0.46, p=0.036

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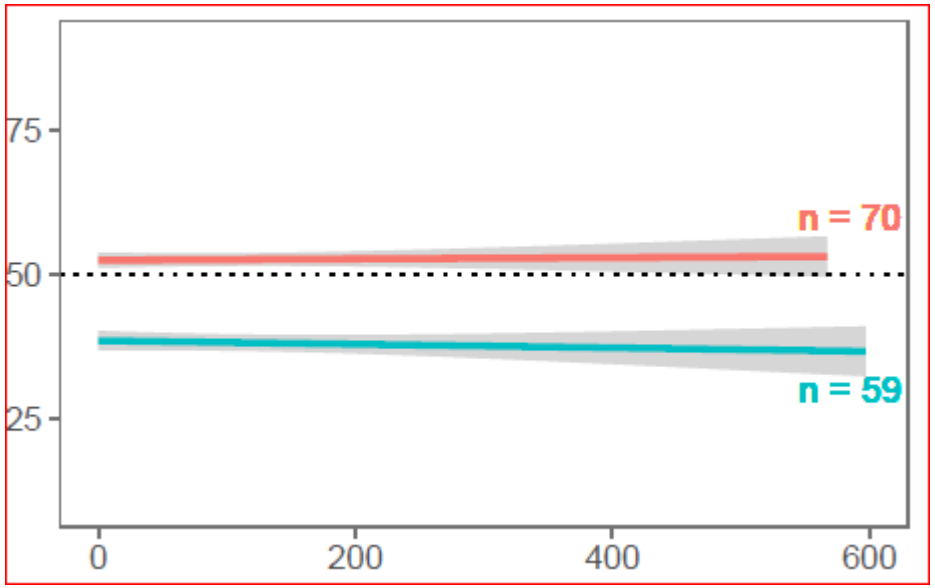
# Peer Relationships

**PARENT**

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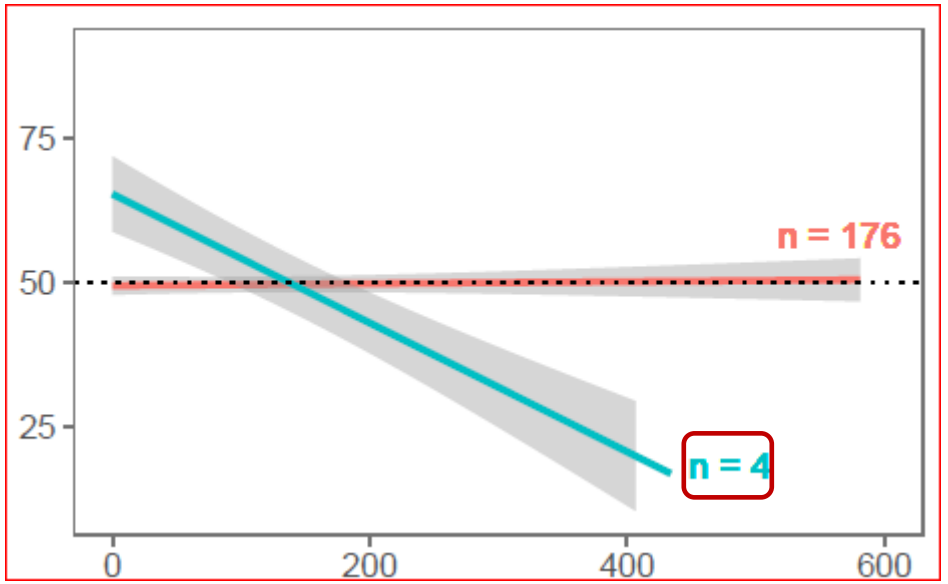


Correlation between change scores of parents and patients: r = 0.183, p=0.4685

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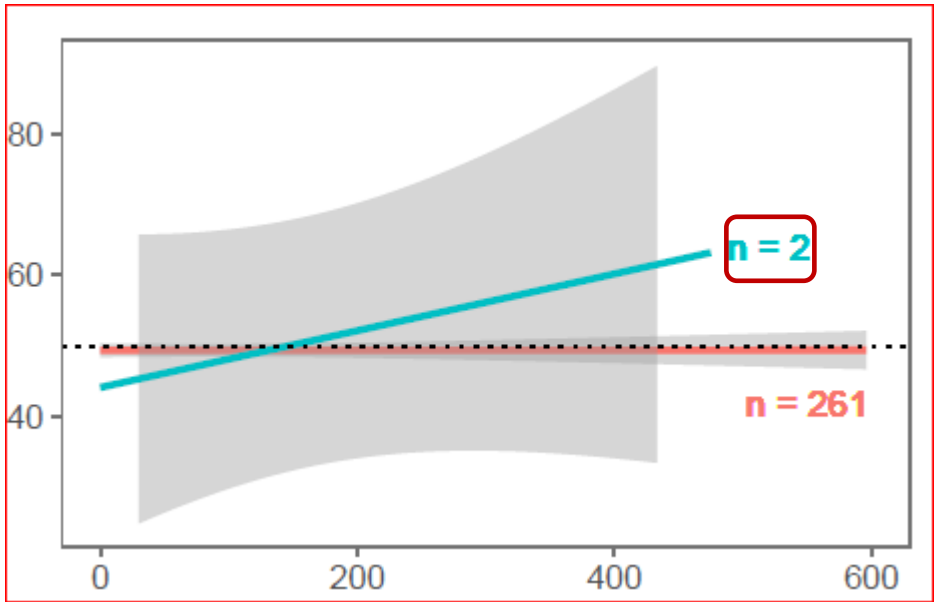
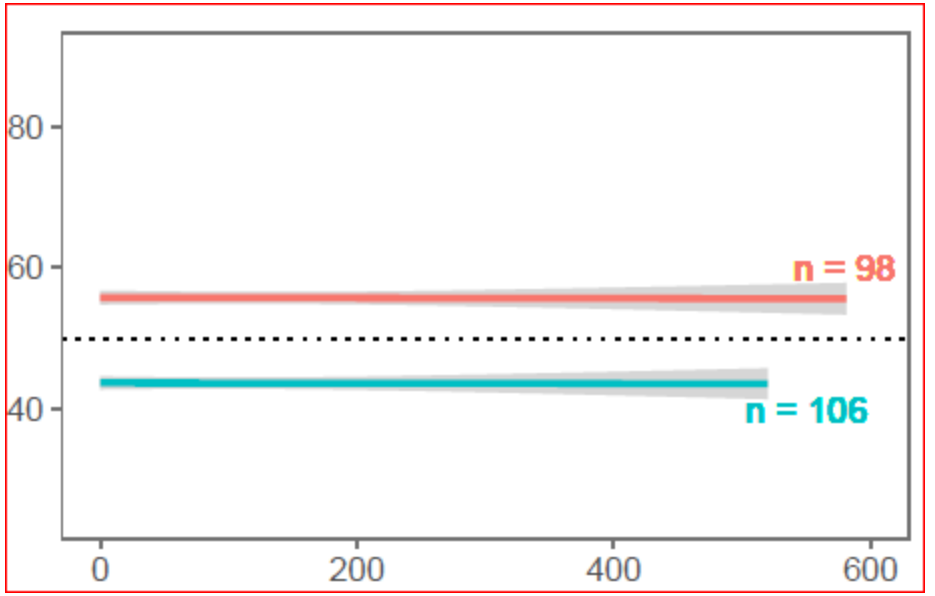
# Cognition

**PARENT**




**PATIENT**




Correlation between change score of parents and patients: r = 0.119, p=0.3557

# Predictors of Pattern (Class) Membership

| Domain | | number of classes [a] | Sample n (by class) | Marital status | Gender (child) | IEP | Parent rated QOL | Initial dx or recurrent | Number of tx received | Length (chemo) | Length (radiation) | PSR | Age (parent) | Age (child) | Years since dx | Years since last tx |
|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|---|
| Anxiety | child | 2[a] | 4[a]; 68; 108 | | * | | *** | | | | | | | | * | |
| Depression | child | 2 | 88; 95 | | | | *** | | | * | * | | | | ** | |
| Fatigue | child | 4 | 73;63;54;5 | | | ** | *** | | ** | | | ** | | * | | |
| Mobility | child | 1[a] | 2[a];189 | | | | | | | | | | | | | |
| UE | child | 5 | 17;96;35;26;13 | | | | * | * | * | ** | * | ** | * | *** | *** | ** |
| Peer | child | 1[a] | 174; 4[a] | | | | | * | | | | | | | | |
| Cognition | child | 2 | 95;106 | * | | *** | *** | | | | | | | | | |
| Anxiety | parent | 1[a] | 4[a]; 125 | | | | | | | | | | | | | |
| Depression | parent | 2 | 77; 54 | | | | ** | | | | | | | | | ** |
| Fatigue | parent | 2 | 96;38 | | | | *** | | | | | * | | | | |
| Mobility | parent | 3 | 97; 29; 7 | | * | * | *** | | | | | *** | | ** | | |
| UE | parent | 2 | 34; 97 | | | ** | *** | | | | | ** | *** | *** | | * |
| Peer | parent | 2 | 70; 59 | | | | * | | | | | | | | | |
| Cognition | parent | 1[a] | 259; 2[a] | | | | | | | | | | | | | |

* p<0.05; ** p<0.01; *** p<0.001

UE=Upper Extremity Function; Peer= Peer Relationships; Dx=diagnosis; Tx=treatment

a. A class with a sample size less than 5 was considered trivial and not meaningful.

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# Conclusions

- Linear mixed effects models showed declined patient-reported Cognition and parent-reported Anxiety over time.
- Patient-reported Mobility and Upper Extremity Function predicted patients' survival.
  - Small sample size (death n=24).
- At the individual level, patients and parents showed different patterns of changed PROMIS scores over time across all domains, except *depressive symptoms*.
  - Significant factors differentiating class membership were identified, which were domain specific.
  - Different predictors were found between parents and patients

*Study 3:*

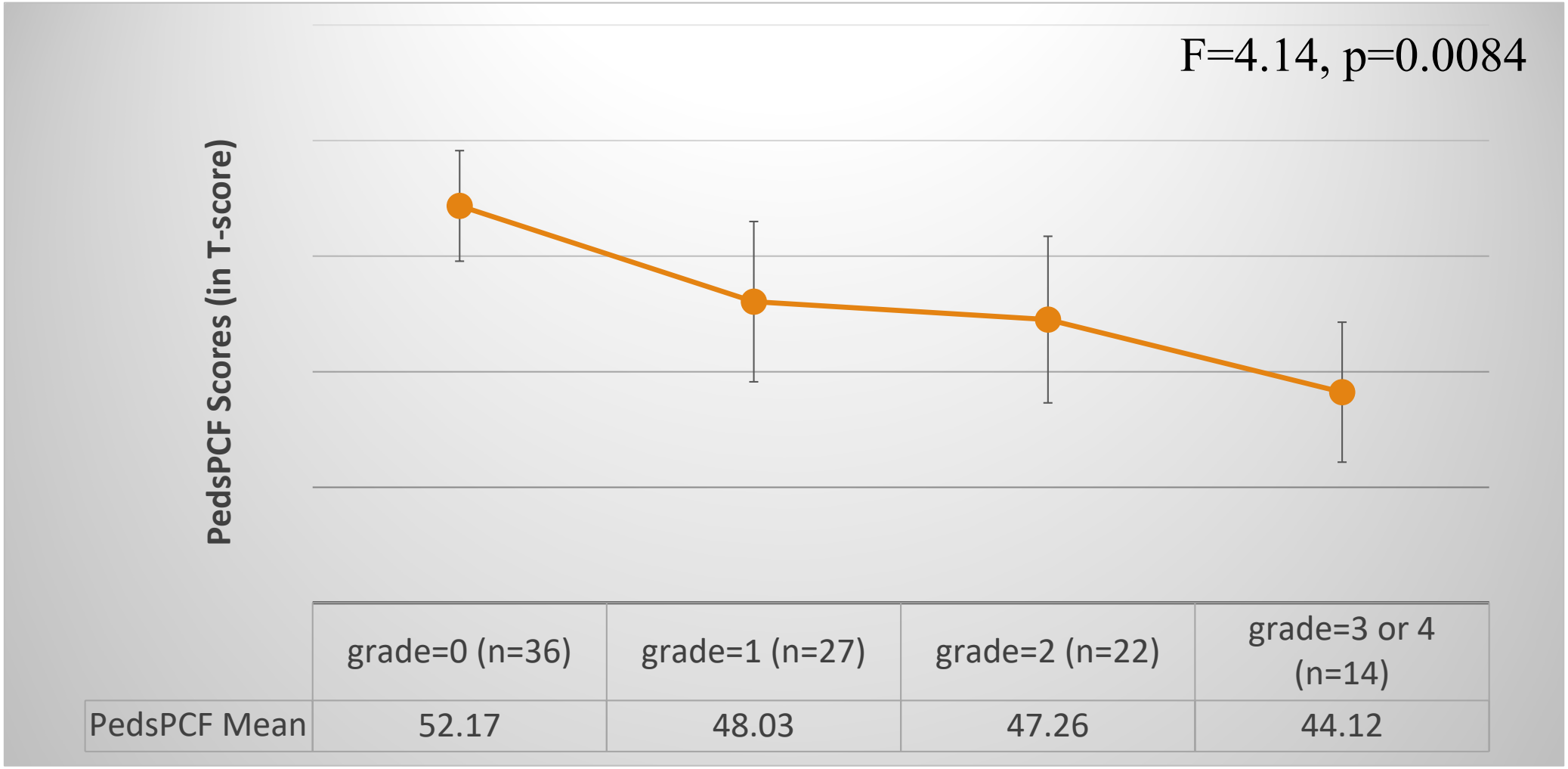
# *Association between the pediatric PROMIS Cognition and Leukoencephalopathy of Children with Brain Tumors*

Lai et al (2017). doi: 10.1007/s11136-017-1583-8. Epub 2017 Apr 26. PMID: 28447250 PMCID: PMC5658265

# Leukoencephalopathy Grades

| Grade | Criteria |
|---|---|
| 0 | normal, injury not perceived |
| 1 | • mild generalized white matter signal abnormality;<br>• minimal or mild generalized volume loss; or<br>• signal abnormality/damage limited to 1 lobe of involvement. |
| 2 | • moderate generalized white matter signal abnormality;<br>• moderate generalized volume loss; or<br>• signal abnormality/damage limited to 2 lobes of involvement. |
| 3 | • severe generalized white matter signal abnormality;<br>• severe diffuse volume loss; or<br>• signal abnormality/damage involving at least 3 lobes |
| 4 | • near complete loss of the white matter volume; or<br>• complete infiltration of the white matter by signal abnormality within the entire hemisphere |

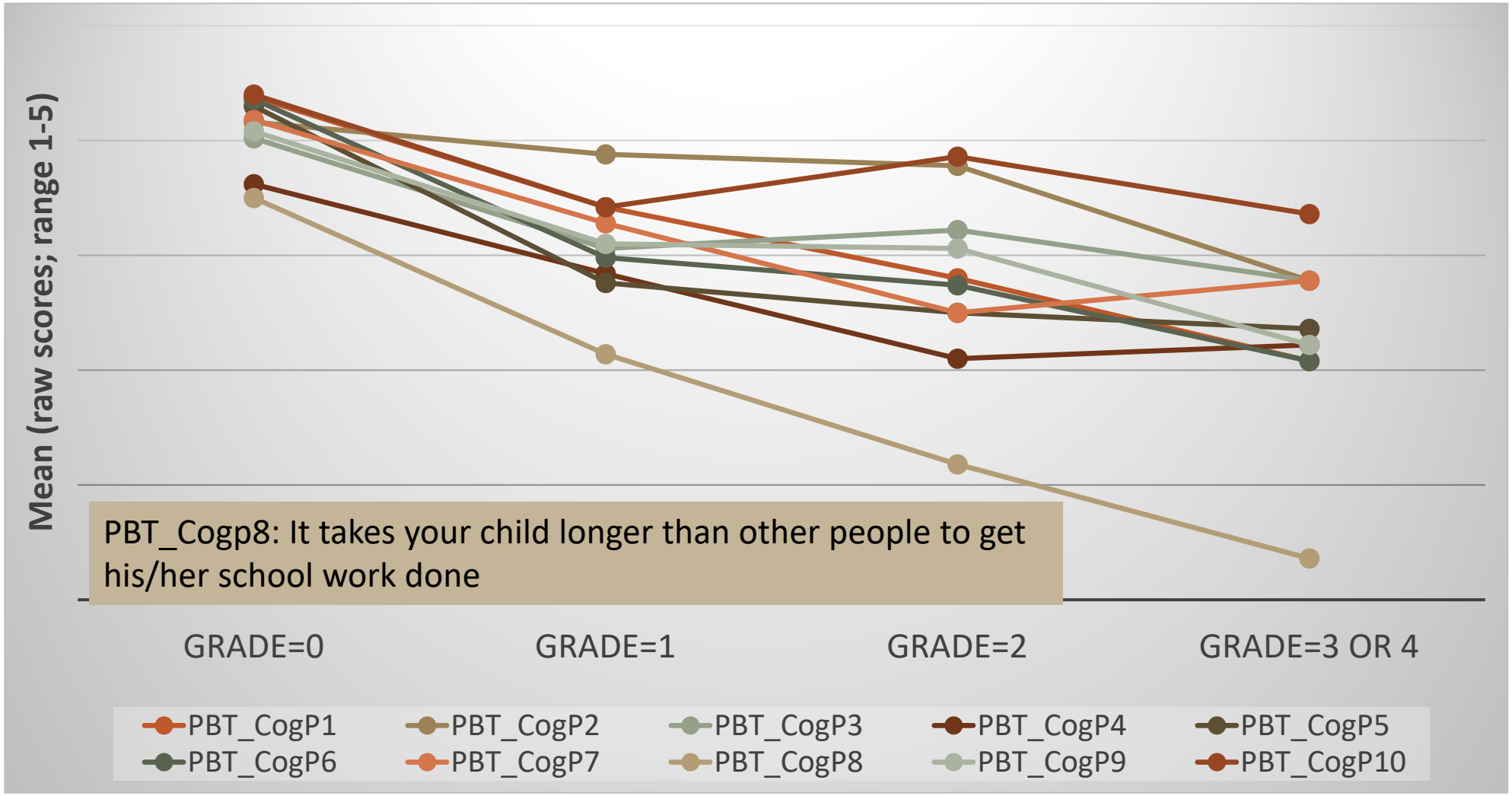
Leukoencephalopathy grade was based on white matter damage and degree of deep white matter volume loss shown on MRI




| | grade=0 (n=36) | grade=1 (n=27) | grade=2 (n=22) | grade=3 or 4 (n=14) |
|---|---|---|---|---|
| PedsPCF Mean | 52.17 | 48.03 | 47.26 | 44.12 |

n = 99; a subsample from the study 1

# Mean comparisons of individual pedsPCF items




n = 99; a subsample from the study 1

# *Study 4: It is feasible to administer CAT in pediatric neuro-oncology clinics?*

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# Results: Time and # of items to complete CAT

| Itm bank | Time (SD)[a] | Number of items administered | | |
|---|---|---|---|---|
| | | Mean (SD)[b] | Min | Max |
| **Anxiety** | 1.38 (1.69) | 9.7 (2.9) | 5 | 13 |
| **Fatigue** | 2.01 (3.96) | 8.7 (2.8) | 5 | 13 |
| **Mobility** | 1.46 (0.98) | 8.1 (3.3) | 5 | 13 |
| **Upper Extremity** | 1.3 (0.97) | 10.4 (2.7) | 5 | 13 |
| **Depression** | 1.31 (2.46) | 8.3 (3.4) | 5 | 13 |
| **Peer relationship** | 1.49 (1.95) | 8.1 (3.2) | 5 | 15 |

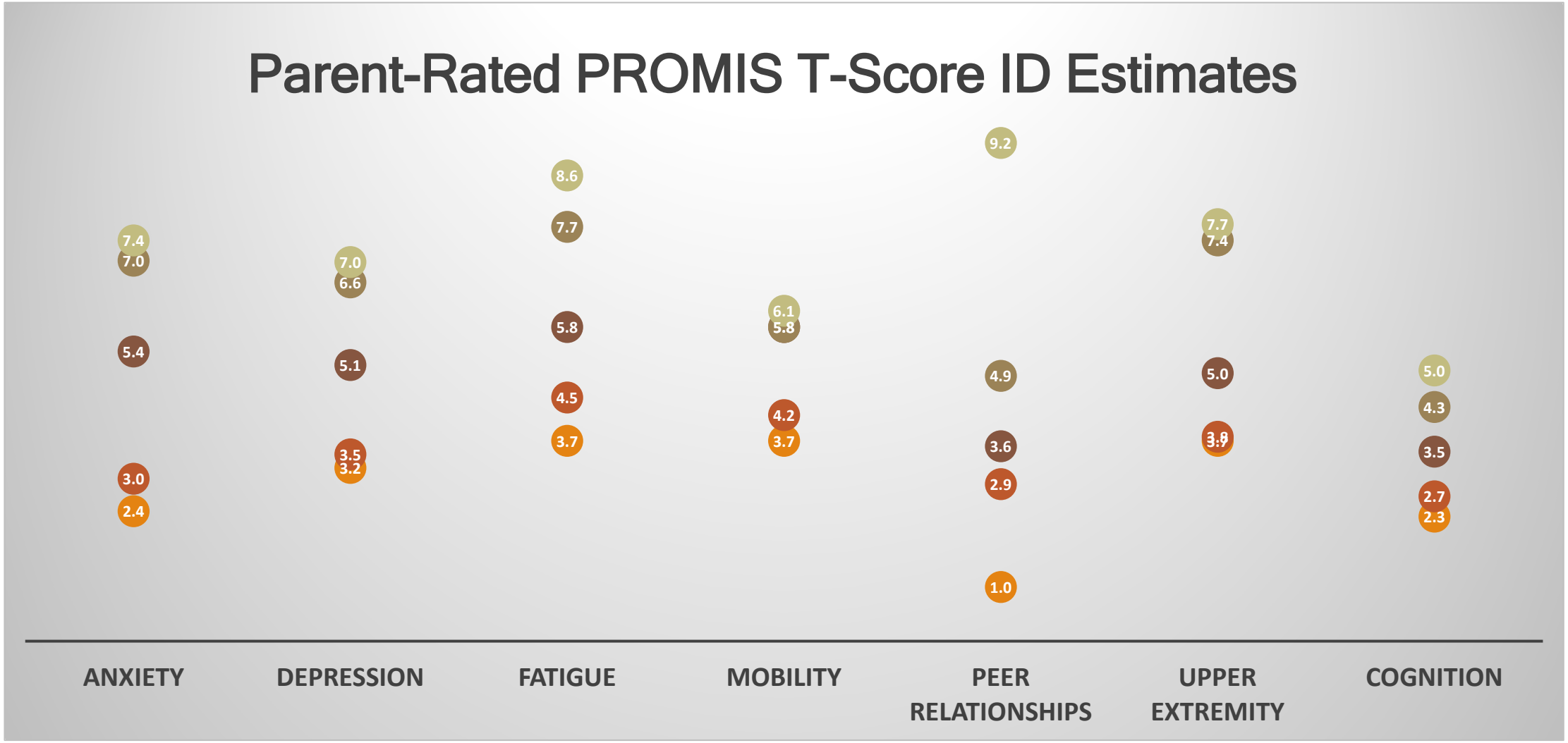
a. Time to complete CAT, in minutes
b. Number of CAT items administered

*(Lai et al, 2017)*

Lai et al. (2017) doi:10.1016/j.jpainsymman.2017.05.008. PMID: 28797854

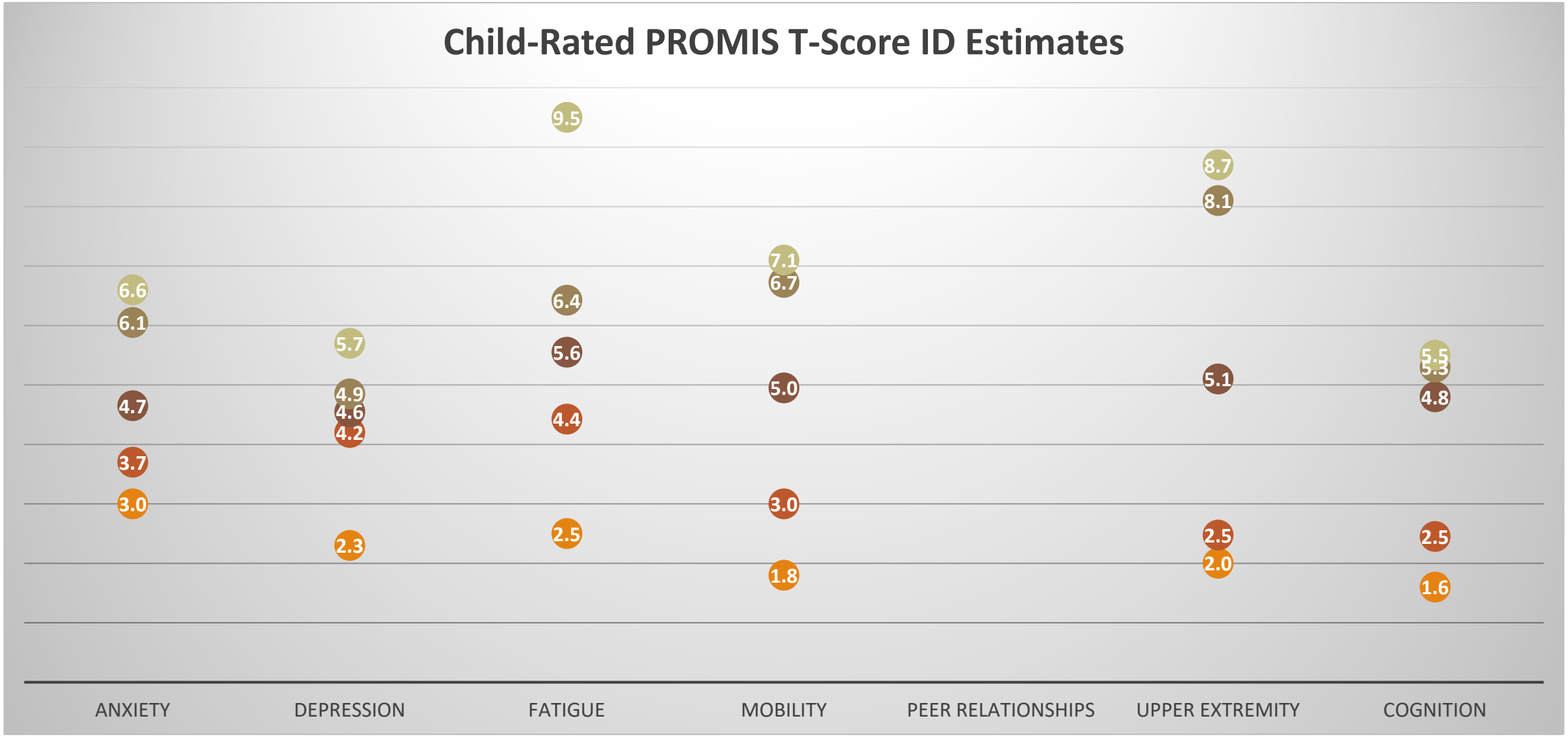
# *Study 5: (Minimally) important differences*

The estimated important differences (IDs) for **parent-rated** T-scores were: Anxiety 3-7 points, Depression, 3.5-6.5 points, Fatigue 4.5-7.5 points, Mobility 4-6 points, Peer Relationships 3-5 points, Upper Extremity Function 4-7.5 points, and Cognition 2.5 – 4.5 points.




*Symbols in figure represent minimum, 1st quartile, median, 3rd quartile, and maximum values for the list of included cross-sectional and longitudinal anchor-based differences*

The estimated important differences (IDs) for **child-rated** T-scores were: Anxiety 4-6 points, Depression, 4-5 points, Fatigue 4.5-6.5 points, Mobility 3-6.5 points, Upper Extremity 2.5-8 points, and Cognition 2.5 - 5.5 points. None of the anchor-based analyses for child-rated Peer Relationships met the criteria for inclusion.

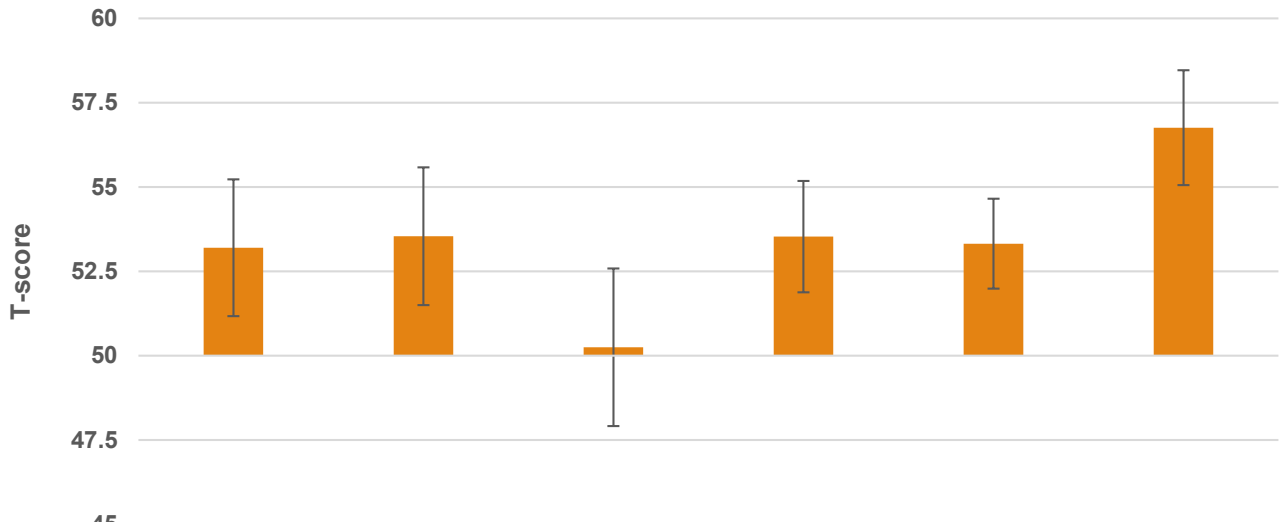



*Symbols in figure represent minimum, 1st quartile, median, 3rd quartile, and maximum values for the list of included cross-sectional and longitudinal anchor-based differences*

# *Children with other chronic conditions such as NF1-associated plexiform neurofibromas*

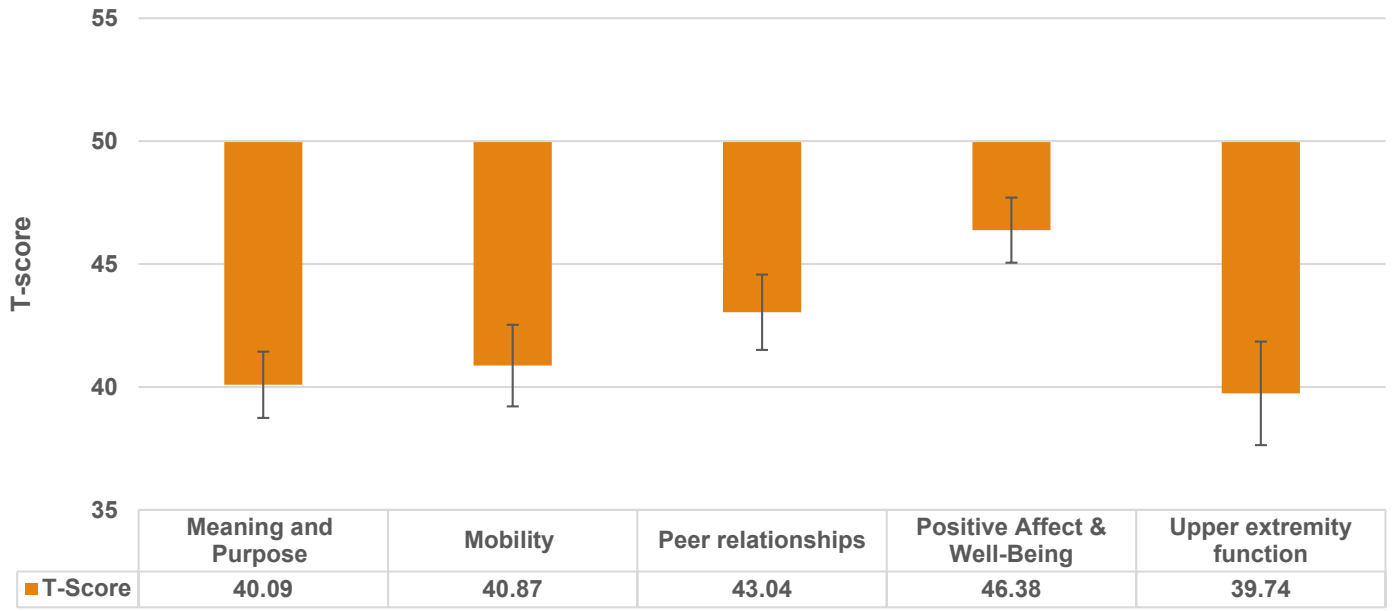
Lai et al (2019) doi: 10.1016/j.jpeds.2018.10.019. Epub 2018 Nov 6. PMID: 30413310

# QOL vs. General Population Norms (mean=50 SD=10)




| | Anxiety | Depressive symptom | Fatigue | Pain | Stigma | Psychological Stress Experiences |
|---|---|---|---|---|---|---|
| T-Score | 53.2 | 53.54 | 50.25 | 53.53 | 53.32 | 56.76 |

# QOL vs. General Population Norms (mean=50 SD=10)



# v.s. PROMIS Global Health

| | Health | Quality of life | Physical health | Mental health | Feel really sad | Have fun with friends | Listen to child's ideas | Get tired easily | Trouble sleeping when Having pain |
|---|---|---|---|---|---|---|---|---|---|
| **Anxiety** | *** | *** | *** | *** | *** | *** | *** | *** | *** |
| **Depressive symptom** | *** | *** | *** | *** | *** | *** | *** | *** | *** |
| **Fatigue** | *** | *** | *** | *** | *** | *** | *** | *** | *** |
| **Stigma** | *** | *** | *** | *** | *** | *** | *** | *** | *** |
| **Pain interference** | *** | *** | *** | *** | *** | *** | *** | *** | *** |
| **Psychological Stress Experiences** | *** | *** | *** | *** | *** | *** | *** | *** | *** |
| **Meaning and Purpose** | *** | *** | *** | *** | *** | *** | *** | * | *** |
| **Mobility** | *** | *** | *** | *** | *** | *** | *** | *** | *** |
| **Peer relationships** | *** | *** | *** | *** | *** | *** | *** | *** | *** |
| **Positive Affect & Well-Being** | ** | *** | *** | *** | *** | *** | *** | ** | *** |
| **Upper extremity function** | *** | *** | *** | *** | *** | *** | *** | *** | *** |

(ANOVA) *** $p<0.001$; ** $p<0.01$; * $0<0.05$

# v.s. Demographic & Clinical Variables

| | Gender | family w/ NF1 besides your child | chronic itch | Pain | age at diagnosis | # of café-au-lait spots | # of plexiform neurofibroma |
|---|---|---|---|---|---|---|---|
| Anxiety | ns | <.0001 | ns | <.0001 | 0.0004 | ns | ns |
| Depressive symptom | ns | <.0001 | ns | <.0001 | <.0001 | ns | 0.0097 |
| Fatigue | ns | <.0001 | ns | <.0001 | 0.0013 | ns | 0.0126 |
| Pain | 0.0023 | <.0001 | ns | <.0001 | 0.0001 | ns | ns |
| Stigma | 0.0123 | <.0001 | ns | <.0001 | 0.0118 | ns | ns |
| Psychological Stress Experiences | ns | <.0001 | ns | <.0001 | 0.0003 | ns | ns |
| Meaning and Purpose | 0.0018 | 0.0249 | ns | <.0001 | <.0001 | 0.031 | ns |
| Mobility | 0.0011 | <.0001 | ns | <.0001 | <.0001 | 0.0151 | ns |
| Peer relationships | ns | 0.0232 | 0.0468 | 0.0022 | ns | ns | ns |
| Positive Affect & Well-Being | ns | 0.0001 | ns | 0.0033 | 0.0154 | ns | ns |
| Upper extremity function | 0.0017 | <.0001 | ns | <.0001 | <.0001 | 0.0134 | ns |

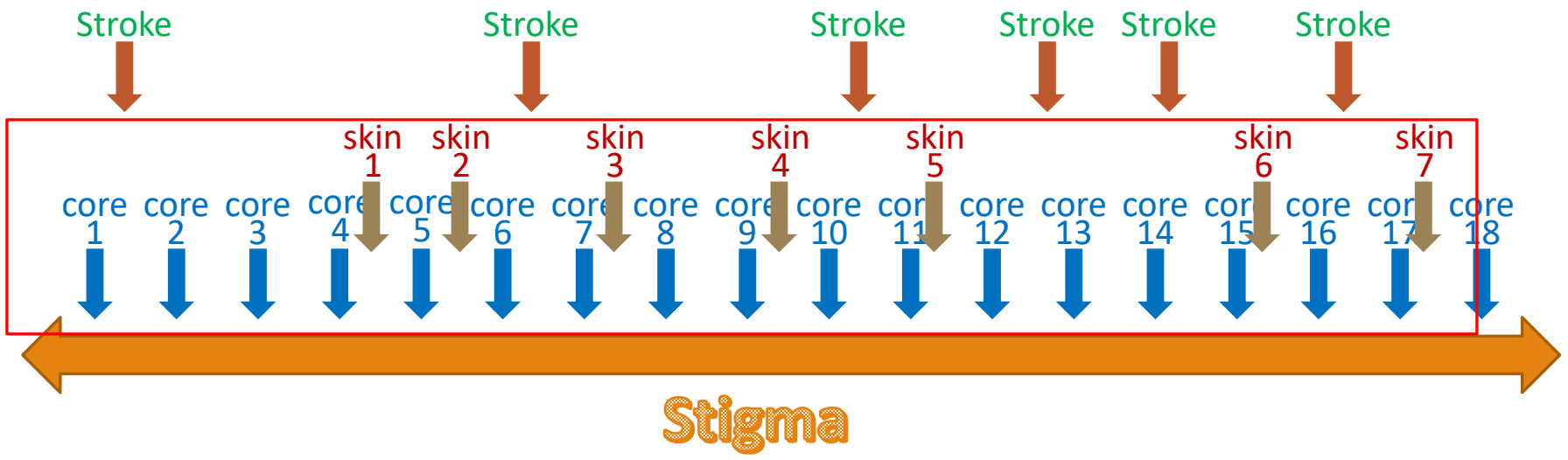
Age at diagnosis: “10-17 years old” vs. “5-9 years old” vs. “Under 5 years old”
# of café-au-lait spots: “No” vs. “Yes: <= 6” vs. “Yes: 6-20 (inclusive)” vs. “Yes: > 20”
# of plexiform neurofibroma(s): “No” vs. “Yes: just one” vs. “Yes: 1-5” vs. “Yes: 5 or more”

# Conclusions

1. Empirical evidences support pediatric PROMIS is a valid measurement system to evaluate symptom burden/health-related quality of life on children with brain tumors.
2. Symptom-based measurement systems can be used on children with various conditions who experience same symptoms such as fatigue, depression etc.
3. National based norms -- common reference group
   - Particularly important for children with rare diseases
   - Core set items + condition specific items
4. The need of individualized, tailored assessment such as PROMIS CATs when monitoring patients' HRQOL across the disease continuum
   - ~ 2 min to complete each CAT – *individualized & tailored*
   - PROMIS is available in Epic 2017 and newer versions – *feasible in clinics*
   - Link to adult measures – *across the lifespan*

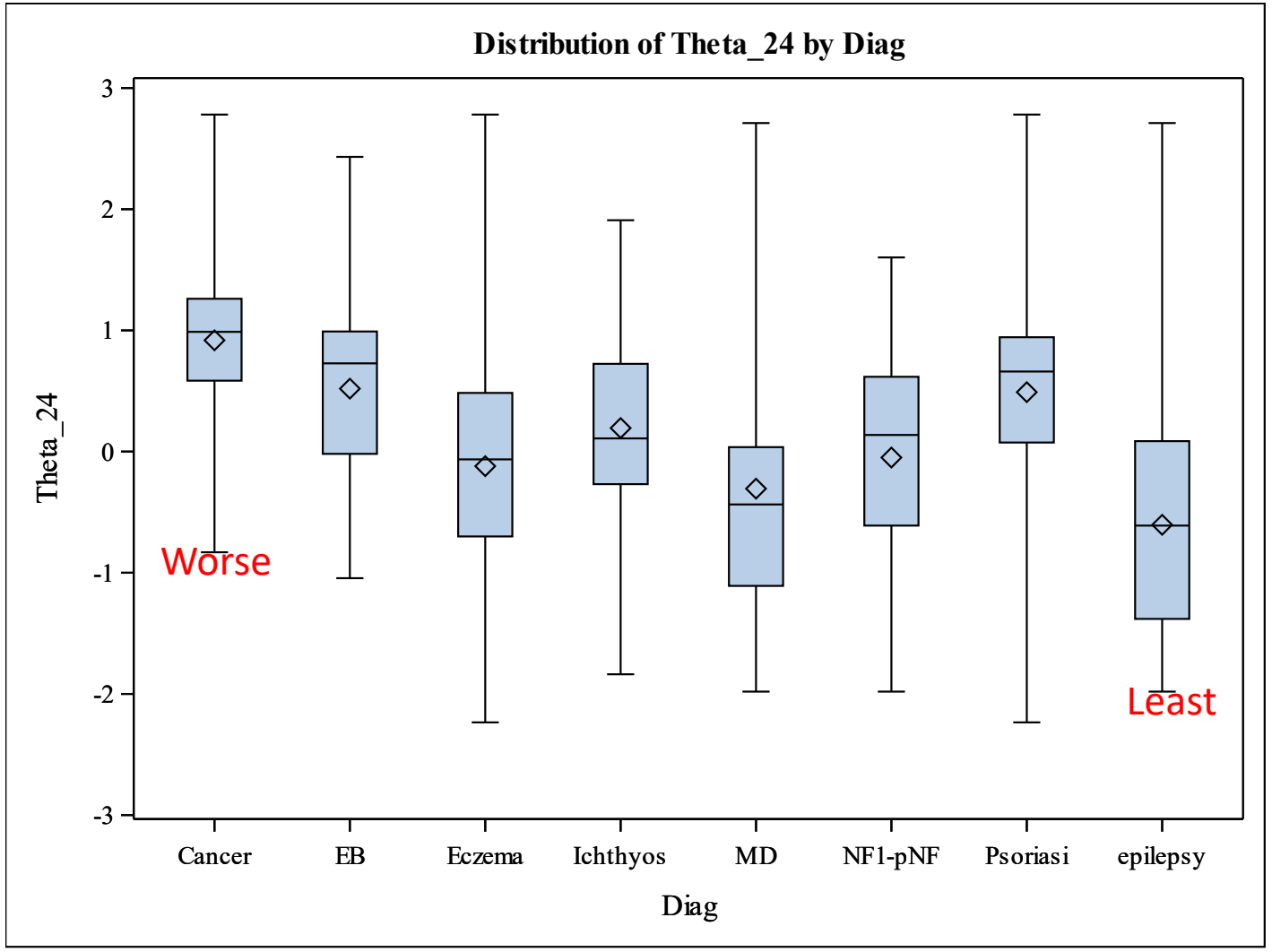
# Using Perceived Stigma as an Example
# Core Stigma Item Set + Condition Specific Items

**Make it possible to compare stigma perceived between patients with stroke vs. skin condition without losing the sensitivity to capture stigma resulted from each condition**




**core + condition specific items --**
**capturing unique condition/disease experiences**

# Perceived Stigma across Conditions




*Neuro-QoL/PEPR example*

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