

Caregiver-Reported Performance Measures in SMA: An Update

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Capacity, Capability and Performance (Holsbeeke 2009)

- Capacity: “what one can do in a standardized, controlled environment”
- Capability: “what one can do in his/her daily environment” – caregiver/self-report
- Performance: “what a person actually does in his/her daily environment” – caregiver/self-report

Capability is not always reflected in capacity measures, and capability does not always translate to typical performance



Performance/Capability Measures

- Clinician, caregiver or self-report
- Perception of performance/participation in a life situation
- Inter-rater variability
- Typically more specific to functional activities , ADL's & leisure activities
- May relate more directly to motor capacity measures

Quality of Life Measures

- Clinician, caregiver or self-report
- Perception of well-being across various domains
- Inter-rater variability
- Multi-dimensional
 - Emotional well being
 - Social well being
 - Material well being
 - Physical well being
 - Self-esteem
 - Self-determination

BOTH ARE OF IMPORTANT IN CAPTURING CHANGE OVER TIME

Examples in SMA

- “I know he’s having a hard time bringing his hand to his head today, but he’s reaching for toys in his stroller more at home.”
- “I wish you saw how fast he pushed his manual wheelchair on the playground yesterday!”
- “She can crawl around on the hardwood floor at home, but this mat makes it harder for her.”
- “Can I send you some videos of how well she’s holding her head up in her stander?”
- “ I can take the cap off of a bottle of water with less help now.”

Performance Measures Used in SMA: Examples

- Egen-Klassifikation Scale -2
(Steffenson 2002, Fagoaga 2013, Alemdaroglu 2014, Emery 2015)
 - Caregiver-reported/clinician-report with paper/pen
 - 10 questions- 0-4 item response (ordinal level); (+) 7 items
 - Non-ambulatory individuals only
 - May be most sensitive to change in SMA during rapid periods of functional loss (i.e. post-operatively)
 - Not specific to developmental age
 - Administration time: 10 minutes
 - Item examples
 - Ability to lift arms over head
 - Amount of support required in standing
 - Overall physical well-being throughout the day

Performance Measures Used in SMA: Examples

- Assessment of Caregiver Experience with Neuromuscular Disease (ACEND) (Matsumoto 2011, Difazio 2015)
 - Caregiver-reported – designed to measure change following orthopedic intervention in neuromuscular disease
 - 7 domains assessing mobility, transfers, self-feeding, time burden, finance, emotion
 - How much help does the child require? (percentage)
 - Several activities combined into one question (rolling, scooting, crawling)
 - Validated in cerebral palsy with Gross Motor Function Classification System (GMFCS) Levels I-V
 - Utilized in Ionis trials

ACEND Item Example

17. Does your child roll, scoot or crawl on the floor?

- Yes, without my help
- Yes, but I need to be present only for safety, to give direction or help with setup
- Yes, but I provide a small amount of physical help (1% to 10%)
- Yes, but I provide some physical help (11 to 49 %)
- Yes, but I provide maximal help (50% to 99%)
- No, I provide total help (100%)

Performance Measures Used in SMA: Examples

- Pediatric Evaluation of Disability Inventory Computer Adaptive Test (PEDI-CAT) (Haley 2012)
 - Caregiver reported outcome measure intended for children with a variety of cognitive, motor and behavioral difficulties
 - Expanded item bank from original PEDI (276 items versus 197 items), measuring mobility, daily activities, social/cognition and responsibility
 - Expanded age range (1-21 years)
 - Reduced caregiver burden by minimizing number of item responses (15/276 items/domain)
 - Improved scoring from dichotomous to interval
 - iPad version and Windows version


PEDI-CAT Item Examples: Daily Activities Domain

PEDICAT for Windows - □ ×

Daily Activities Identification Number: 0222

Pulls open a sealed bag of snack food

- Unable = Can't do, doesn't know how or is too young.**
- Hard = Does with a lot of help, extra time, or effort.**
- A little hard = Does with a little help, extra time or effort.**
- Easy = Does with no help, extra time or effort, or child's skills are past this level.**
- I don't know.**



Previous Next

PEDI-CAT Item Examples: Daily Activities Domain

PEDICAT for Windows - □ ×

Daily Activities Identification Number: b

Puts on and fastens pants


Unable = Can't do, doesn't know how or is too young.

Hard = Does with a lot of help, extra time, or effort.

A little hard = Does with a little help, extra time or effort.

Easy = Does with no help, extra time or effort, or child's skills are past this level.

I don't know.




PEDI-CAT Item Examples: Mobility Domain

PEDICAT for Windows Identification Number: 00

Mobility

Stands for a few minutes. Please do not consider use of walking aids (walker, crutches or canes).

- Unable = Can't do, doesn't know how or is too young.**
- Hard = Does with a lot of help, extra time, or effort.**
- A little hard = Does with a little help, extra time or effort.**
- Easy = Does with no help, extra time or effort, or child's skills are past this level.**
- I don't know.**



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PEDI-CAT Item Examples: Mobility Domain

PEDICAT for Windows - □ ×

Mobility Identification Number: b

Uses manual wheelchair to move from room to room in home

- Unable = Can't do, doesn't know how or is too young.**
- Hard = Does with a lot of help, extra time, or effort.**
- A little hard = Does with a little help, extra time or effort.**
- Easy = Does with no help, extra time or effort, or child's skills are past this level.**
- I don't know.**



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PEDI-CAT Item Examples: Mobility Domain

PEDICAT for Windows _ □ ×

Mobility Identification Number: b

Steps up and down curbs. Please do not consider use of walking aids (walker, crutches or canes).


Unable = Can't do, doesn't know how or is too young.

Hard = Does with a lot of help, extra time, or effort.

A little hard = Does with a little help, extra time or effort.

Easy = Does with no help, extra time or effort, or child's skills are past this level.

I don't know.



PEDI-CAT Item Examples: Mobility Domain

PEDICAT for Windows - □ ×

Mobility Identification Number: b

Goes up and down stairs by crawling or scooting on bottom

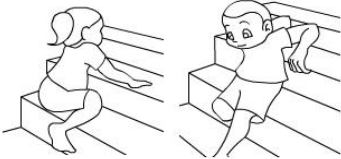
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PEDI-CAT Scoring

- Age percentiles and T-scores based on normative standardization sample for 21 age groups
 - T Score (0-100); Mean of 50 +/- 10; (scores >30 = “normal range”)
- Scaled (criterion scores) based on data from the normative and disability samples
 - Based on an estimate of placement along hierarchical scale within each domain
 - Currently on a 20-80 scale metric (allows for additional items to be added)

Some PEDI-CAT Publications

- Dumas (2012): Discriminant validity (disability), test-reliability, administration time
- Dumas (2012): Discriminant validity for walking aid versus manual wheelchairs
- Dumas (2012): Concurrent validity and reliability with PEDI
- Kramer (2015): Reliability, Validity and Utility in Autism Spectrum Disorders
- Dumas (2015): Concurrent validity with Alberta Infant Motor Scales (2015)
- Frazier (2016): Validation and feasibility in CP clinic
- Fragala-Pinkham (2016): Responsiveness to change in pediatric inpatient-rehabilitation setting



PEDI-CAT for SMA Option Goals

(PNCRN effort with support from the SMA Foundation)

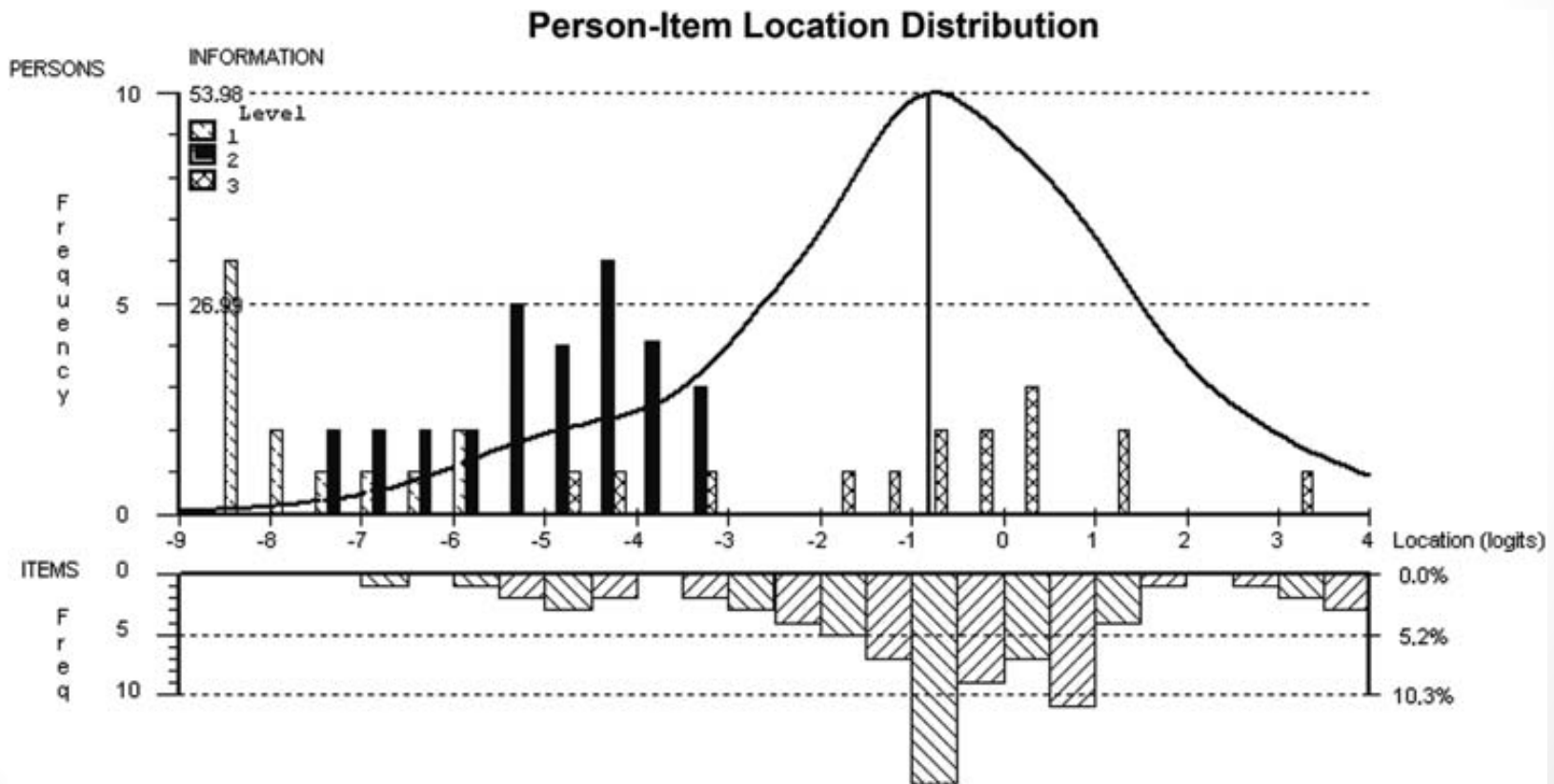
1. Administration feasibility in clinic
2. Test response and test information functions in SMA Type I, II and III (Rasch analysis)
3. Item maps identifying gaps across all 3 types, as well as across content areas
4. Identify areas for improvement from expert clinicians and caregivers
5. Ultimate goal: Develop a PEDI-CAT SMA Option for use in natural history and clinical trials

PEDI-CAT for SMA Updates

Muscle & Nerve (2016)

- 58 full item bank (paper versions) and 62 CAT versions administered to caregivers or young adults with SMA across 3 sites
 - **Type I (n=13; mean age= 4.35 y (0.44 - 10.04))**
 - **Type II (n=33; mean age = 8.21 y (1.1 – 23))**
 - **Type III (n=16; mean age = 13.02 y (4.77 - 21.68))**
- Rasch analysis results: The PEDI-CAT works best for:
 - Type III Mobility and Daily Activities (upper extremity tasks) as well Daily Activities for Type II
 - Less difficult items need to be added to increase the validity and sensitivity for Type I and Type II

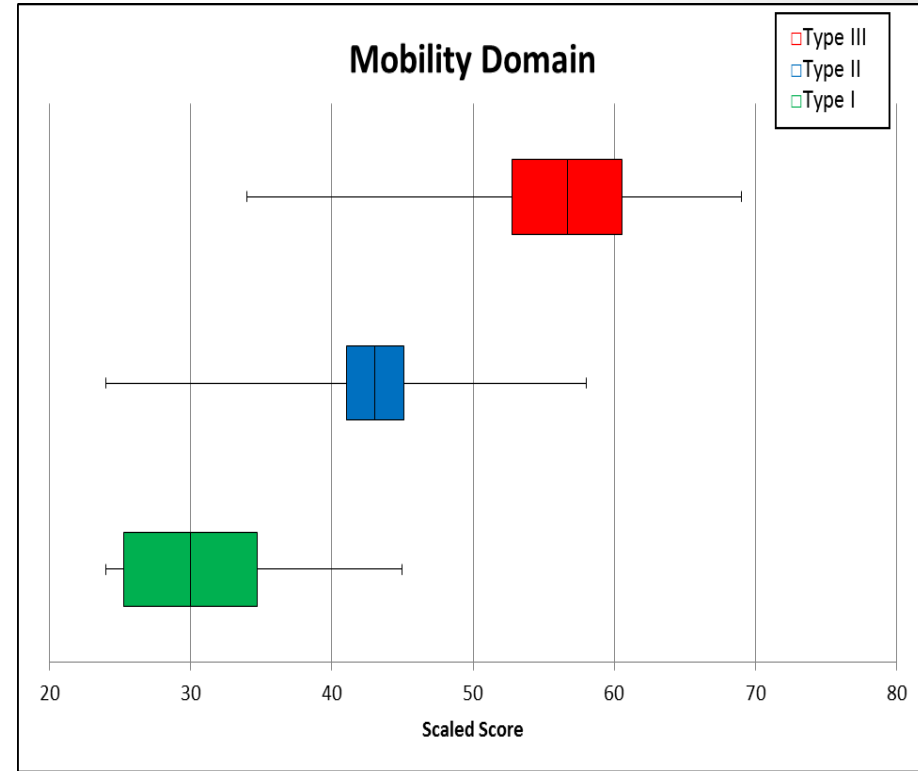
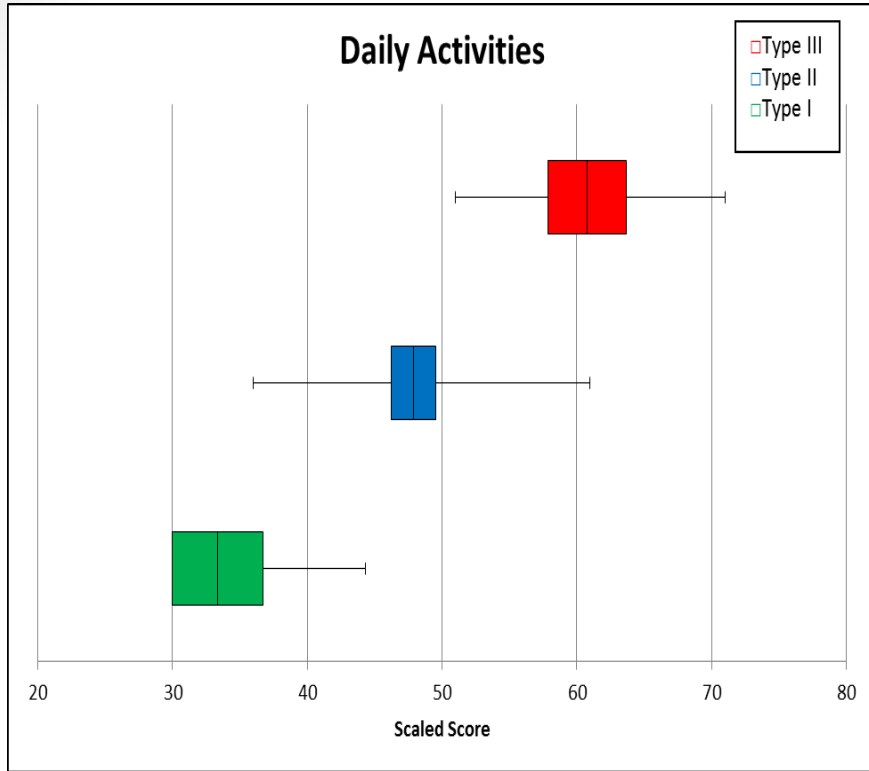
Rasch Analysis: Mobility Domain



PEDI-CAT for SMA Updates

Cure SMA 2015 (Poster)

- Feasibility of administration in clinic
 - Combined administration time (Daily Activities and Mobility)
 - Mean = 6 minutes, Mode = 5 minutes, Range = 2-13 minutes
- Discriminative properties (n = 70 caregivers)
 - Scaled scores discriminate across Types I, II, & III
- Evaluative properties (n = 70 caregivers)
 - Standard scores indicate presence of disability across SMA in the domains involving physical functioning in Types I, II & II
- Social/Cognitive Function (n=22/70 Type II/III) = within normal range for age



Daily Activities:

($F=99.9$, $p<0.001$)

Type I (32.0, SD 5.0)

Type II (47.9, SD 5.2)

Type III (60.8, SD 5.8)

Mobility:

($F=42.4$, $p<0.00$)

Type I (29.9, SD 8.1),

Type II (44.05, SD 7.1),

Type III (56.8, SD 8.8)

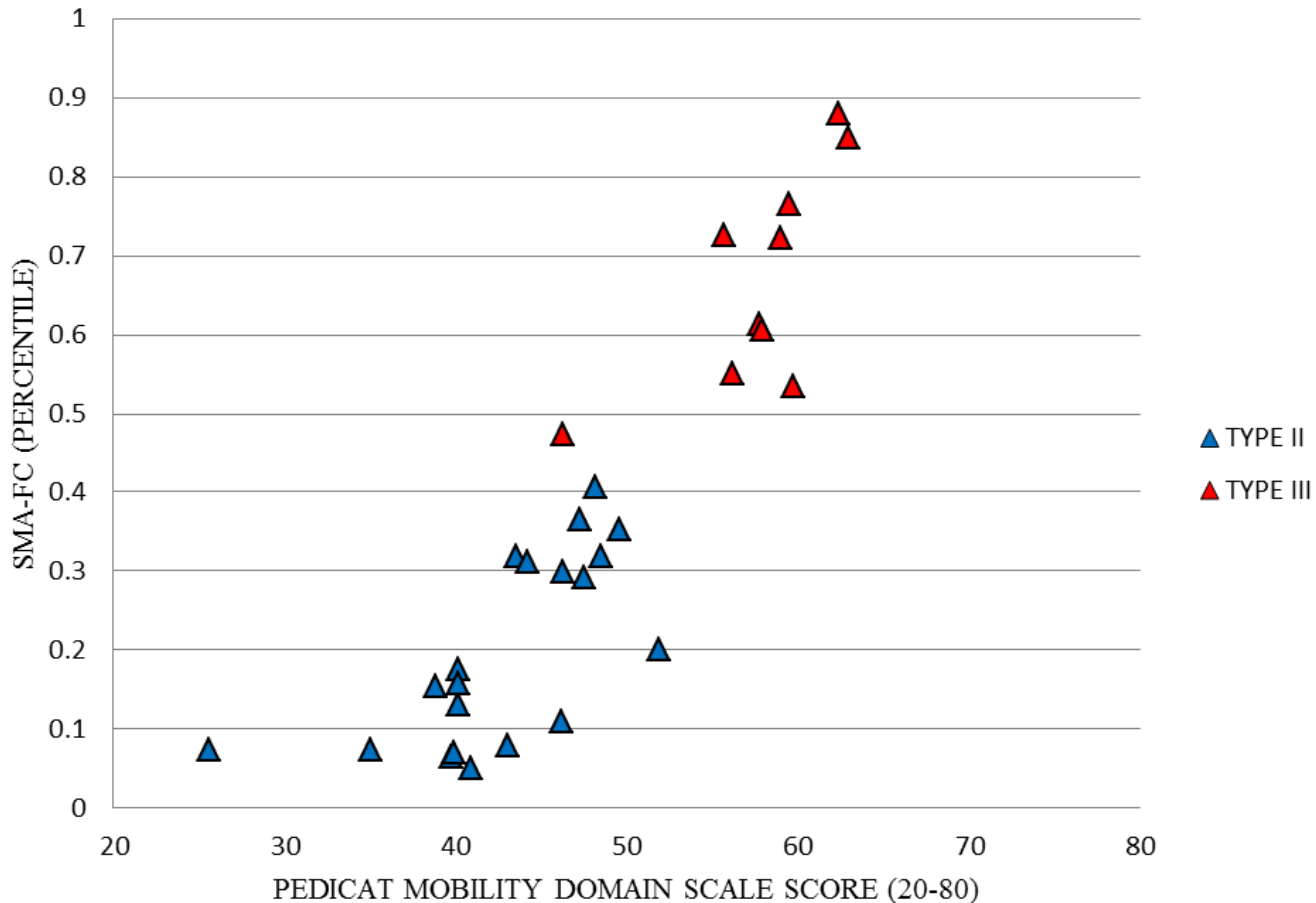
PEDI-CAT for SMA Updates

Cure SMA 2016 (Poster)

- Relationships between PEDI-CAT and capacity measures
- HFMSE (n = 10 SMA III, 29 SMA II; mean age = 10.45y; 2 - 24.7y)
 - Mobility and Daily Activities (r = 0.85, r = 0.80; p<0.0001, p<0.0001)
- ULM (n = 5 SMA III, 20 SMA II; mean age 11.03y; 3.8y - 24.7y)
 - Mobility and Daily Activities (r = 0.77, r = 0.76; p<0.0001, p<0.0001)
- 6MWT (n = 9 SMA III; mean age 12.82y; 6.6y – 17.7y)
 - Mobility Domain: (r = 0.82 p = 0.006)
 - Daily Activities (r = -0.10 p = 0.98)
- SMA-FC (n = 10 SMA III, 19 SMA II; mean age 12.03y; 2.9y – 24.7y)
 - Mobility and Daily Activities (r = 0.89, r = 0.78; p<0.0001, p<0.0001)

Pasternak, A., Fragala-Pinkham, M., Glanzman, A., Sideridis, G., Montes, J., Dunaway, S., Quigley, J., Mirek, L., Salazar, R., S., Pandya, LaMarca, N., Cruz, R., (Szlag) Fournier, H., Madabusi, L., Harrington, T., Videon, N., Martens, W., McDermott, M., Chiriboga, C., Finkel, R., Tennekoon, G., De Vivo, D., Darras, B. (June 2016). *Relationships between the Pediatric Evaluation of Disability Inventory Computer Adaptive Test (PEDI-CAT) and Disease-Specific Capacity Measures for Spinal Muscular Atrophy (SMA)*. Paper presented at Cure SMA 2016, Anaheim, CA.

SMA-FC AND PEDICAT MOBILITY DOMAIN

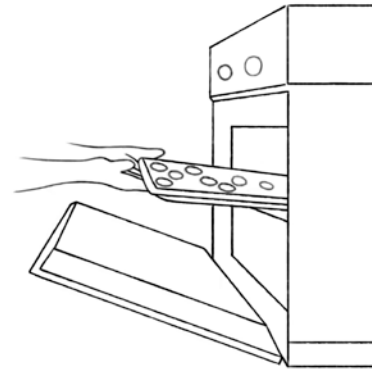
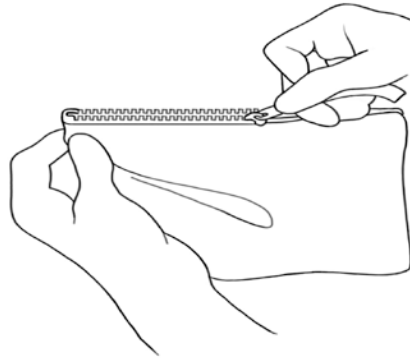
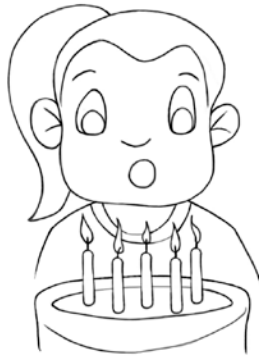


$$r = 0.89; p < 0.0001$$

PEDI-CAT for SMA Updates

- 4 expert clinician focus groups (in-person/phone)
- Informal patient and caregiver interviews (in-person/electronic email)
 - 150 new items and illustrations
 - Power wheelchair mobility, feeding items for Type I and weak Type II, head control items.....
- Ongoing:
 - Cognitive testing/interviewing of new items prior to validation
 - Continued collection of current PEDI-CAT (CAT version only) in clinic focusing on Type III SMA (to validate with 6MWT)
 - Investigating language interpretations
 - Recommend using PEDI-CAT as is, given progress observed in Ionis trials

PEDI-CAT for SMA: New item examples (Daily Activities)



PEDI-CAT for SMA: New item examples (Mobility)



